

PROSPECTUS

6,974,248 Shares of Common Stock



This prospectus relates to the offer and resale from time to time of up to 6,974,248 shares (the “Shares”) of common stock, par value \$0.0001 per share (“Common Stock”), of Lexeo Therapeutics, Inc., a Delaware corporation (the “Company”), by the selling stockholders identified in this prospectus, including their transferees, pledgees or donees or their respective successors (the “Selling Stockholders”). The Shares consist of (i) 6,278,905 shares which were issued and sold to the Selling Stockholders on March 13, 2024 (the “Closing Date”) in a private placement (the “Private Placement”) pursuant to a common stock purchase agreement among us and such Selling Stockholders dated March 11, 2024 (the “Purchase Agreement”) and (ii) 695,343 shares of Common Stock held by the Selling Stockholders as of March 11, 2024. Concurrently with the Purchase Agreement, we entered into a registration rights agreement (the “Registration Rights Agreement”) with the Selling Stockholders, and we are registering the Shares being offered hereunder pursuant to such registration rights agreement on behalf of the Selling Stockholders, to be offered and sold by them from time to time.

The Selling Stockholders may sell any, all or none of the securities and we do not know when or in what amount the Selling Stockholders may sell their securities hereunder following the date of this prospectus. The Selling Stockholders may sell the securities described in this prospectus in a number of different ways and at varying prices. We provide more information about how the Selling Stockholders may sell their securities in the section titled “*Plan of Distribution*” appearing elsewhere in this prospectus.

We will not receive any of the proceeds from the sale of the securities by the Selling Stockholders. We will pay the expenses associated with registering the sales by the Selling Stockholders other than any underwriting discounts and commissions, as described in more detail in the section titled “*Use of Proceeds*” appearing elsewhere in this prospectus.

Of the 6,974,248 shares of Common Stock that may be offered or sold by Selling Stockholders identified in this prospectus, 695,343 of those shares (the “Lock-Up Shares”) are subject to certain lock-up restrictions pursuant to contractual agreements further described in the section titled “*Certain Relationships and Related Person Transactions*” appearing elsewhere in this prospectus.

Our Common Stock is listed on The Nasdaq Global Market (“Nasdaq”) under the symbol “LXEO”. On April 11, 2024, the last quoted sale price for our Common Stock as reported on Nasdaq was \$13.06 per share.

We are an “emerging growth company” and a “smaller reporting company” as defined under the federal securities laws, and, as such, may elect to comply with certain reduced public company reporting requirements for this prospectus and for future filings. See “Prospectus Summary—Implications of Being an Emerging Growth Company and a Smaller Reporting Company.”

Investing in our securities involves a high degree of risk. Before buying any securities, you should carefully read the discussion of the risks of investing in our securities in “[Risk Factors](#)” beginning on page 13 of this prospectus.

You should rely only on the information contained in this prospectus or any prospectus supplement or amendment hereto. We have not authorized anyone to provide you with different information.

Neither the Securities and Exchange Commission nor any state securities commission has approved or disapproved of these securities or determined if this prospectus is truthful or complete. Any representation to the contrary is a criminal offense.

Prospectus dated April 12, 2024

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You should rely only on the information contained in this prospectus or in any applicable prospectus supplement prepared by us or on our behalf. Neither we nor the Selling Stockholders have authorized any other person to provide you with different information. If anyone provides you with different or inconsistent information, you should not rely on it. Neither we nor the Selling Stockholders are making an offer to sell these securities in any jurisdiction where the offer or sale is not permitted. You should assume that the information appearing in this prospectus is accurate only as of the date on the front cover of this prospectus. Our business, financial condition, results of operations and prospects may have changed since that date.

This prospectus is part of a registration statement on Form S-1 that we filed with the Securities and Exchange Commission (the "SEC") using the "shelf" registration process. Under this shelf registration process, the Selling Stockholders hereunder may, from time to time, sell the shares offered by them described in this prospectus. We will not receive any proceeds from the sale by such Selling Stockholders of the securities offered by them described in this prospectus.

We may also provide a prospectus supplement or post-effective amendment to the registration statement to add information to, or update or change information contained in, this prospectus. You should read both this prospectus and any applicable prospectus supplement or post-effective amendment to the registration statement together with the additional information to which we refer you in the section of this prospectus titled "*Where You Can Find Additional Information.*"

The Lexeo design logo and the Lexeo mark appearing in this prospectus are the property of Lexeo Therapeutics, Inc. Trade names, trademarks and service marks of other companies appearing in this prospectus are the property of their respective holders. We have omitted the ® and TM designations, as applicable, for the trademarks used in this prospectus.

We obtained the industry, statistical and market data in this prospectus from our own internal estimates and research as well as from industry and general publications and research, surveys and studies conducted by third parties. All of the market data used in this prospectus involve a number of assumptions and limitations, and the

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sources of such data cannot guarantee the accuracy or completeness of such information. While management is responsible for the accuracy of such data and believes that each of these studies and publications is reliable, the industry in which we operate is subject to a high degree of uncertainty and risk due to a variety of important factors, including those described in the section titled "*Risk Factors*." These and other factors could cause results to differ materially from those expressed in the estimates made by third parties and by us.

SPECIAL NOTE REGARDING FORWARD-LOOKING STATEMENTS

This prospectus contains forward-looking statements that involve risks and uncertainties. We make such forward-looking statements pursuant to the safe harbor provisions of the Private Securities Litigation Reform Act of 1995 and other federal securities laws. All statements other than statements of historical facts contained in this prospectus, including statements regarding our future results of operations and financial position, business strategy, development plans, planned preclinical studies and clinical trials, future results of clinical trials, expected research and development costs, regulatory strategy, timing and likelihood of success, as well as plans and objectives of management for future operations, are forward-looking statements. In some cases, you can identify forward-looking statements by terms such as “may,” “will,” “should,” “expect,” “plan,” “anticipate,” “could,” “intend,” “target,” “project,” “estimate,” “believe,” “estimate,” “predict,” “potential” or “continue” or the negative of these terms or other similar expressions intended to identify statements about the future. Forward-looking statements contained in this prospectus include, without limitation, statements about:

- the timing, progress and results of our preclinical studies and clinical trials of our product candidates, including statements regarding the timing of initiation and completion of studies or trials and related preparatory work, the period during which the results of the trials will become available and our research and development programs;
- the timing of our planned investigational new drug, or IND, submissions, initiation of planned clinical trials and timing of expected clinical results for LX2006, LX1001, LX2020, if applicable, and our other future product candidates;
- the timing of any submission of filings for regulatory approval of and our ability to obtain and maintain regulatory approvals for LX2006, LX1001, LX2020 and any other product candidates;
- the impact of public health crises (such as COVID-19) and other adverse global economic conditions on our operations and the potential disruption in the operations and business of third-party manufacturers, contract research organizations, or CROs, other service providers, and collaborators with whom we conduct business;
- our ability to identify patients with the diseases treated by our product candidates, and to enroll patients in trials;
- our expectations regarding the size of the patient populations, market acceptance and opportunity for and clinical utility of our product candidates, if approved for commercial use;
- our manufacturing capabilities and strategy, including the scalability and commercial viability of our manufacturing methods and processes;
- our reliance on third party manufacturing partners to comply with significant regulations with respect to manufacturing our products;
- our expectations regarding the scope of any approved indication for LX2006, LX1001, LX2020 or any other product candidate;
- our ability to successfully commercialize our product candidates, if approved;
- our ability to leverage our platform to identify and develop future product candidates;
- our estimates of our expenses, ongoing losses, future revenue, capital requirements and our need for or ability to obtain additional funding before we can expect to generate any revenue from product sales;
- our ability to establish or maintain collaborations or strategic relationships and any expected benefits related thereto;
- our ability to identify, recruit and retain key personnel;
- our reliance upon intellectual property licensed from third parties and our ability to obtain such licenses on commercially reasonable terms or at all;

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- our ability to protect and enforce our intellectual property position for our product candidates, and the scope of such protection;
- our financial performance;
- our competitive position and the development of and projections relating to our competitors or our industry;
- our estimates regarding future revenue, expenses and needs for additional financing;
- the impact of laws and regulations; and
- our expectations regarding the time during which we will be an emerging growth company under the Jumpstart Our Business Startups Act of 2012, or JOBS Act.

We caution you that the foregoing list does not contain all of the forward-looking statements made in this prospectus.

We have based these forward-looking statements largely on our current expectations and projections about our business, the industry in which we operate and financial trends that we believe may affect our business, financial condition, results of operations and prospects, and these forward-looking statements are not guarantees of future performance or development. These forward-looking statements speak only as of the date of this prospectus and are subject to a number of risks, uncertainties and assumptions described in the section titled “*Risk Factors*” and elsewhere in this prospectus. Because forward-looking statements are inherently subject to risks and uncertainties, some of which cannot be predicted or quantified and some of which are beyond our control, you should not rely on these forward-looking statements as predictions of future events. Although we believe that we have a reasonable basis for each forward-looking statement contained in this prospectus, the events and circumstances reflected in our forward-looking statements may not be achieved or occur and actual results could differ materially from those projected in the forward-looking statements. Except as required by applicable law, we do not plan to publicly update or revise any forward-looking statements contained herein, whether as a result of any new information, future events or otherwise.

In addition, statements that “we believe” and similar statements reflect our beliefs and opinions on the relevant subject. These statements are based upon information available to us as of the date of this prospectus, and while we believe such information forms a reasonable basis for such statements, such information may be limited or incomplete, and our statements should not be read to indicate that we have conducted an exhaustive inquiry into, or review of, all potentially available relevant information. These statements are inherently uncertain, and you are cautioned not to unduly rely upon these statements.

PROSPECTUS SUMMARY

This summary highlights, and is qualified in its entirety by, information contained elsewhere in this prospectus. This summary does not contain all of the information you should consider before investing in our Common Stock. You should read this entire prospectus carefully, especially the sections titled “Risk Factors” and “Management’s Discussion and Analysis of Financial Condition and Results of Operations” and our financial statements and the related notes thereto appearing elsewhere in this prospectus, before making an investment decision. As used in this prospectus, unless the context otherwise requires, references to “we,” “us,” “our,” “the Company,” and “Lexeo” refer to Lexeo Therapeutics, Inc.

Overview

We are a clinical stage genetic medicine company dedicated to transforming healthcare by applying pioneering science to fundamentally change how disease is treated. By taking aim at the underlying genetic cause of the devastating diseases we target, we seek to create substantial positive impact and reduce the overwhelming burdens placed on people receiving treatment, their caregivers, and healthcare systems. Our current pipeline consists of candidates targeting patient populations that place significant burden on society and are most amenable to our genetic medicine approach.

Our most advanced cardiovascular product candidate, LX2006 for the treatment of patients with Friedreich’s ataxia, or FA, cardiomyopathy, is currently being evaluated in SUNRISE-FA, an ongoing Phase 1/2 clinical trial. We have observed an increase in frataxin protein expression in the hearts of three patients that have undergone cardiac biopsies across cohort 1 (n=1) and cohort 2 (n=2), and we expect to report additional interim data from this trial in mid-2024. Our second most advanced cardiovascular product candidate, LX2020 for the treatment of arrhythmogenic cardiomyopathy, or ACM, caused by mutations in the *PKP2* gene, referred to as PKP2-ACM, received investigational new drug, or IND, clearance from the U.S. Food and Drug Administration, or FDA, in July 2023. LX2020 received Fast Track and Orphan Drug designations from the FDA in December 2023. We expect to dose the first patient in a Phase 1/2 clinical trial in the first half of 2024 and provide an interim data readout from cohort 1 in the second half of 2024.

Our lead Alzheimer’s disease product candidate, LX1001, for the treatment of *APOE4* homozygous patients with Alzheimer’s disease, is in an ongoing Phase 1/2 trial. In December 2022, we reported that we observed an increase in expression levels of the protective protein, *APOE2*, in the first dose cohort and a consistent trend towards improvement in core Alzheimer’s disease biomarkers in the first dose cohort. We completed enrollment of the trial in the fourth quarter of 2023 and expect to report additional interim data from all cohorts in the Phase 1/2 clinical trial in the second half of 2024.

We are targeting diseases that have seen limited penetration of precision medicine, which we define as medications that treat the underlying molecular mechanism of a disease, and where we believe there is significant opportunity for gene therapy to play a role as a key therapeutic option. We believe the specific indications we are initially targeting, FA cardiomyopathy, PKP2-ACM and *APOE4*-associated Alzheimer’s disease, are highly amenable to gene therapy, where administration of a single dose has the potential to either restore loss-of-function or minimize gain-of-function mutations by treating the underlying genetic cause of the disease. Although few precision medicines are currently approved for the treatment of cardiovascular conditions or Alzheimer’s disease, recent approvals by the FDA suggest a willingness to approve new precision medicines based on biomarkers and functional endpoints. Together with improved diagnostics and increased testing, these developments may offer one of the most substantial opportunities for the uptake of precision medicines in the global pharmaceutical marketplace.

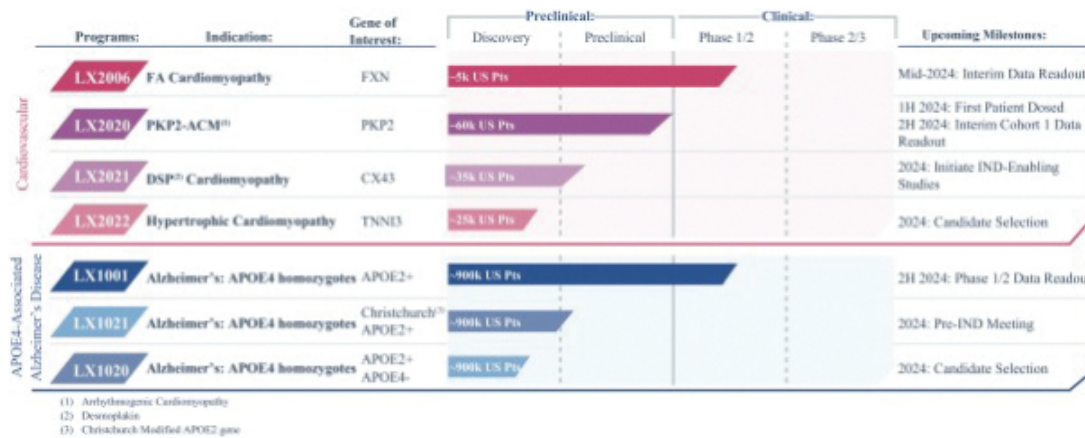
Each of our gene therapy candidates utilizes the vector construct, dose and route of administration that we believe will result in the most favorable biodistribution and safety profile for our product candidate for each

disease. Our most advanced cardiovascular and APOE4-associated Alzheimer’s disease programs use the AAVrh10 vector due to its high transduction efficiency in both myocardial cells and neurons, potential for lower toxicity given the opportunity to utilize lower doses compared to other well-established AAV serotypes, and low pre-existing immunity.

By specifically tailoring our technological approach to each targeted disease, we believe we can optimize our programs to achieve the highest likelihood of having therapeutic impact. We target genetically defined indications in specific sub-groups of patients that offer the potential to demonstrate therapeutic impact through improvement in functional endpoints or biomarkers, have high unmet need and large market opportunities, have established proof-of-concept in relevant preclinical models, and have organized patient advocacy groups and identifiable patient populations. In addition to targeting cardiovascular diseases and APOE4-associated Alzheimer’s disease that we believe can be addressed by our current approach utilizing AAVrh10, we have ongoing discovery efforts to identify next-generation vector technologies with the best potential therapeutic profile. Finally, we continuously seek to bolster our pipeline through relationships with academic institutions, providing us access to cutting edge genetic medicines research which will include not only AAV gene therapy but also other potential therapeutic payload types and non-viral delivery systems. In August 2023, we announced a strategic investment from Sarepta Therapeutics, Inc. to explore collaboration opportunities within our preclinical cardiovascular pipeline.

Our pipeline

Utilizing a stepwise, capital-efficient development approach, we are leveraging early proof-of-concept functional and biomarker data to advance a deep and diverse pipeline of cardiovascular and APOE4-associated Alzheimer’s disease programs for larger-rare and prevalent indications. We retain exclusive worldwide development and commercialization rights to all of our product candidates and programs.



Cardiovascular programs

We are developing a number of disease-modifying gene therapy candidates to treat larger-rare cardiovascular diseases that have significant unmet need and no approved treatments that address the underlying genetic cause of the disease. These programs include:

- LX2006 is an AAVrh10-based gene therapy candidate designed to intravenously deliver a functional frataxin, or FXN, gene for the treatment of FA cardiomyopathy. FA cardiomyopathy is the most

common cause of mortality in patients with FA and affects approximately 5,000 patients in the United States. LX2006 is designed to promote the expression of the protein frataxin to restore normal mitochondrial function and energy production in myocardial cells. In preclinical studies, LX2006 demonstrated improvement in cardiac function and survival in a severe *FXN* knockout mouse model. Similarly, in a partial *FXN* knockout mouse model, LX2006 was observed to restore cardiac function and reverse disease abnormalities of FA cardiomyopathy. LX2006 is currently being evaluated in an open-label, ascending dose Phase 1/2 clinical trial in patients with FA cardiomyopathy and we have observed an increase in frataxin protein expression in the hearts of three patients that have undergone cardiac biopsies across cohort 1 (n=1) and cohort 2 (n=2). We expect to report additional interim data from this trial in mid-2024. The FDA has granted Rare Pediatric Disease designation and Orphan Drug designation to LX2006 for the treatment of FA.

- *LX2020* is an AAVrh10-based gene therapy candidate designed to intravenously deliver a functional *PKP2* gene to cardiac muscle for the treatment of PKP2-ACM. *PKP2* mutations are associated with approximately 75% of all genetic cases of ACM, and we estimate they affect approximately 60,000 patients in the United States. *PKP2* mutations can cause replacement of heart muscle with fibrotic tissue and fatty deposits, and severe abnormal heart rhythms, or arrhythmias, that cause cardiac dysfunction and can result in sudden cardiac death. LX2020 is designed to increase desmosomal *PKP2* protein levels, reassemble desmosomes and restore myocardial cell function. In our preclinical studies, using a genetic mouse model of ACM harboring a *PKP2* mutation that recapitulates the phenotype of PKP2-ACM, LX2020 resulted in fewer arrhythmias and increased survival. We received IND clearance for LX2020 from the FDA in July 2023 and Fast Track and Orphan Drug designations from the FDA in December 2023. We expect to dose the first patient in a Phase 1/2 clinical trial in the first half of 2024 and provide an interim data readout from cohort 1 in the second half of 2024.
- *LX2021* is a gene therapy candidate we are developing to intravenously deliver the coding sequence for the functional connexin 43, or Cx43, protein for a group of inherited cardiac muscle disorders associated with a high risk of sudden death, including ACM and certain forms of dilated cardiomyopathy. We believe restoring the Cx43 protein can potentially treat multiple genetic causes of ACM because the cardiac loss of Cx43 is a molecular deficit generally observed in all ACM patient populations. Our LX2021 program is initially targeting Desmoplakin, or DSP, cardiomyopathy, a distinct form of ACM as well as a certain form of dilated cardiomyopathy, with a prevalence that may be as high as 4% of all inherited dilated cardiomyopathies and 4% of all ACMs, impacting up to approximately 35,000 patients in the United States. We plan to initiate IND-enabling studies for LX2021 in 2024.
- *LX2022* is a gene therapy candidate we are developing to intravenously deliver a functional *TNNI3* gene to myocardial cells to treat a distinct form of hypertrophic cardiomyopathy, or HCM, due to mutations in the *TNNI3* gene. Mutations in the *TNNI3* gene often result in left ventricular hypertrophy and restrictive cardiomyopathy, leading to arrhythmias and heart failure. With an estimated prevalence of 1 in 500 people in the United States, HCM is one of the most common forms of genetic cardiomyopathy caused by mutations that affect the cardiac sarcomere in approximately 75% of cases. It is estimated that as many as 25,000 patients in the United States and 34,000 patients in the European Union are affected by HCM caused by mutations in the *TNNI3* gene. We plan to complete candidate selection for LX2022 in 2024.

APOE4-associated Alzheimer's programs

We are developing a portfolio of approaches to treat the genetics underlying Alzheimer's disease. These programs include:

- *LX1001* is an AAVrh10-based gene therapy candidate designed to deliver into the cerebrospinal fluid, or CSF, an *APOE2* gene for the treatment of *APOE4* homozygous patients with Alzheimer's disease.

Alzheimer's disease is the leading cause of cognitive decline in late adult life and characterized by complex underlying pathology in the central nervous system, or CNS. Individuals homozygous for *APOE4*, an allele of the gene *APOE*, are approximately 15 times more likely to develop Alzheimer's disease than the general population, and it is estimated that there are 900,000 *APOE4* homozygous patients with Alzheimer's disease in the United States alone. Conversely, individuals homozygous for the *APOE* allele *APOE2* are 40% less likely to develop Alzheimer's disease than the general population. This and other evidence suggest that *APOE2* may play a neuroprotective role. *LX1001* is designed to express the protective *APOE2* gene in the CNS of *APOE4* homozygous patients in order to halt or slow the progression of Alzheimer's disease. *LX1001* is being evaluated in an ongoing open-label, dose-escalation Phase 1/2 clinical trial. In the first dose cohort in the trial, we observed a consistent trend towards improvement in Alzheimer's disease CSF biomarkers, such as total tau and phosphorylated tau. We have also observed expression of the protective *APOE2* protein in all patients in the first dose cohort with follow-up data. *LX1001* has been granted Fast Track designation by the FDA for the treatment of patients with early Alzheimer's disease who are *APOE4* homozygous to slow disease progression. We completed enrollment in the trial in the fourth quarter of 2023 and expect to report additional interim data from all cohorts in the Phase 1/2 clinical trial in the second half of 2024.

- *LX1021* is a gene therapy candidate we are developing to deliver a Christchurch mutation-modified *APOE2* allele for the treatment of *APOE4* homozygous patients with Alzheimer's disease. The Christchurch mutation has been recognized to protect individuals against Alzheimer's disease even in the presence of significant amyloid pathology. The mechanism of this protection may relate to the fact that *APOE*, in the presence of the Christchurch mutation, binds poorly to heparan sulfate proteoglycans, which are molecules found on the surface of neurons that may inhibit the spread of tau between cells. We believe this approach has the potential to enhance the protective effect of *APOE2* in homozygous *APOE4*-associated Alzheimer's disease. We plan to hold a pre-IND meeting with the FDA in 2024 to guide the next stage of development for this program.
- *LX1020* is a gene therapy candidate we are developing to deliver both the protective *APOE2* allele and microRNA, or miRNA, to suppress *APOE4* for the treatment of *APOE4* homozygous patients. We believe delivery of *APOE2* with concurrent suppression of *APOE4* will achieve a higher degree of conversion to the *APOE4/E2* heterozygous profile, which should lead to greater therapeutic effect. We plan to complete candidate selection in 2024.

Our approach

Our integrated modular approach enables us to optimize our strategy to pursue larger-rare and prevalent genetically defined indications in specific sub-groups of patients, and is comprised of our technology approach, our precision medicine focus, our disease area strategy, our manufacturing approach and academic collaborations.

Our technology approach

We believe that our approach to technology has the potential to confer the following advantages over existing gene therapy technologies:

- *High Transduction Efficiency and Biodistribution.* The AAVrh10 vector has been shown to be capable of transducing myocardial cells based on preclinical research, and it has been shown to be capable of transducing neurons based on results from preclinical studies and our ongoing clinical trials. We believe these results demonstrate that among currently available, commonly used serotypes, AAVrh10 is an efficient vector for delivery and expression of transgenes for the treatment of the cardiovascular diseases that we are currently targeting and *APOE4*-associated Alzheimer's disease.

- *Reduced Toxicity.* The cardiac tropism of AAVrh10 may allow our gene therapy candidates to be systemically administered at lower doses than many other AAV-based therapies targeting cardiovascular or other systemic diseases.
- *Reduced Pre-Existing Neutralizing Antibodies.* Treatments leveraging vector serotypes to which humans have pre-existing immunity tend to be less effective. Among the naturally occurring and commonly used AAV serotypes, AAVrh10 has been shown in preclinical studies to have among the lowest levels of pre-existing neutralizing antibodies.
- *Optimized Expression.* We are collaborating with our academic partners to develop novel solutions to optimize the potential therapeutic efficacy of our product candidates.

Our precision medicine focus

We believe a precision medicine focus for cardiovascular diseases and APOE4-associated Alzheimer's disease represents a compelling opportunity and the next frontier in expanding the impact of genetic medicines across therapeutic areas:

- *Precision genetic medicines target the underlying cause of the disease.* Next-generation precision based genetic medicines offer the opportunity to directly target the mechanisms underlying cardiovascular disease and Alzheimer's disease.
- *Significant unmet need in cardiovascular disease and APOE4-associated Alzheimer's disease.* More than 30 million adults have been diagnosed and approximately 655,000 Americans die yearly from heart disease. Additionally, we believe there are approximately 900,000 APOE4 homozygous patients in the United States, and current treatment options are likely to be less effective for this subpopulation of patients.
- *Improved landscape of regulatory precedents utilizing cardiac and CNS biomarkers.* Biomarkers can help accelerate development timelines, reduce patient attrition, and reduce the overall cost of drug development by providing an early read on potential efficacy and therapeutic impact.
- *Improved diagnostics expand market opportunity.* Implementation of genetic testing has the potential to help identify patients, including those who are asymptomatic or at a higher likelihood of developing the disease being evaluated or who respond less favorably to currently approved treatments.

Our disease area strategy

Our cardiovascular and APOE4-associated gene therapy programs are designed to have the following characteristics:

- *Indications that may be effectively treated by gene therapy.* We select targets that correspond to populations with a specific genetic profile and clearly defined disease phenotype.
- *Indications with the potential to demonstrate early evidence of meaningful clinical benefit.* We pursue clearly defined biomarkers and functional endpoints that can provide early proof-of-mechanism and inform clinical development decisions, including the potential to seek accelerated approval pathways.
- *Present opportunity to address high unmet medical need.* We are focused on genetically defined cardiovascular diseases and APOE4-associated Alzheimer's disease where there are no currently approved treatments or where we believe our therapeutic candidates will have a meaningful improvement relative to existing standards of care.
- *Significant market opportunity.* We pursue indications with significant commercial opportunities beyond those typically associated with gene therapy companies targeting rare monogenic diseases. Our current focus is on targets that impact larger-rare disease or prevalent disease patient populations.
- *Targets that have established proof-of-concept.* We have leveraged our relationships with academic institutions including Weill Cornell Medical College, or Weill Cornell Medicine, and the University of

California, San Diego, or UCSD, to in-license product candidates with established proof-of-concept in relevant preclinical models that closely resemble the clinical phenotype we are pursuing.

- *Targeted disease areas best treated by optimal delivery technologies.* Our current gene therapy candidates utilize the AAVrh10 vector due to its tropism for the heart and its observed tolerability in clinical trials for CNS disorders. We will pursue the optimal technology to address the diseases of interest for future indications while ensuring sufficient preclinical or clinical validation for any novel approaches.
- *Readily accessible patients.* Our goal is to accelerate patient recruitment for our clinical trials and increase the likelihood of commercial success of our potential products by focusing on diseases with established patient advocacy groups and university researchers who maintain registries of potentially eligible patients. Where possible, we leverage existing natural history studies to better define target patient phenotypes associated with the disease and may utilize these natural history datasets as a control group in future studies.

Our manufacturing approach

We are developing gene therapy candidates for larger-rare and prevalent disease patient populations that require a high-quality process that can produce vectors in relatively large quantities while utilizing traditional biologics manufacturing infrastructure. We utilize a baculovirus/SF9 expression system to manufacture our gene therapy candidates. Our manufacturing platform is designed to infect SF9 cells at high densities in suspension cell culture with both an AAVrh10 and baculovirus containing the transgene. The output is coupled with a chromatography-based purification process which allows for efficient AAV purification, resulting in higher yields and fewer empty AAV capsids than traditional plasmid HEK adherent cell culture approaches.

Traditional adherent HEK manufacturing approaches, which use plasmid transfection to produce viral vectors, are based on mammalian-derived cell lines and cellular components that replicate in mammalian cells. We believe our process has an improved safety profile over these manufacturing approaches for the following reasons: through our sponsored next-generation PacBio and Illumina sequencing analyses performed by one of our clinical research organizations, we have observed reduced incorporation of non-transgene DNA plasmid DNA impurities, from 15% observed in certain adherent HEK systems to 0.2% in our process; our cellular components are non-replicating in mammalian cells; we have eliminated potentially immunogenic or toxic animal-derived proteins; and our cells are grown under serum-free conditions, leading to reduced risk of contamination from animal-derived products.

We believe our manufacturing process enables us to efficiently pursue our goal of targeting larger-rare and prevalent patient populations. Historically, manufacturing challenges largely driven by the quantity of vector required to pursue large commercial opportunities have limited the utility of gene therapy for large patient populations. We believe our proprietary SF9 baculovirus process will allow us to produce vectors at the necessary scale to support the patient populations we are targeting at a cost-of-goods profile similar to what the pharmaceutical marketplace has seen with biologics.

Academic collaborations

Our foundational science stems from partnerships and exclusive licenses with leading academic laboratories at Weill Cornell Medicine and UCSD, two preeminent institutions on the cutting edge of gene therapy research. Of note, our Chief Scientific Advisor, Ronald G. Crystal, M.D., has sponsored 14 cleared gene therapy IND applications across multiple disease areas, and researchers at UCSD led the discovery efforts of a cardiovascular gene therapy program that is expected to move into late-stage clinical trials. We will continue to draw on the scientific expertise provided by these partnerships while evaluating new opportunities for complementary research and development with other academic collaborators.

Our strategy

Our company is purpose built to amplify genetic medicine’s potential for empowering individuals by treating the underlying cause of genetic disease. The key elements of our strategy to achieve this vision are to:

- Focus our AAV-based gene therapy candidates in areas of high unmet need and with substantial potential for societal impact and commercial opportunity, such as genetically defined cardiovascular diseases and APOE4-associated Alzheimer’s disease.
- Advance a deep and diverse pipeline that includes candidates that are designed to address both larger rare and more prevalent patient populations, prioritizing conditions most likely to benefit from our therapies.
- Pursue a staged, capital-efficient approach for advancing programs through clinical development and regulatory approval.
- Utilize a unified, high-quality manufacturing platform that can quickly respond at scale to high impact opportunities.
- Pursue next-generation genetic medicine technologies that can enhance our capabilities and expand our impact on patients.
- Leverage and expand upon our partnerships and exclusive licenses with world-class academic institutions.
- Build a fully integrated genetic medicine company and selectively evaluate strategic opportunities to maximize the impact of our pipeline.

Risks associated with our business

Our business is subject to numerous risks and uncertainties, including those highlighted in the section titled “*Risk Factors*” immediately following this prospectus summary. The following is a summary of the principal risks we face, among others:

- we have incurred significant losses since our inception, and we expect to incur significant net losses for the foreseeable future and may not be able to achieve or sustain revenue or profitability in the future;
- we have a limited operating history, have not completed any clinical trials, and have no products approved for commercial sale;
- if we are unable to raise capital when needed, we could be forced to curtail our planned operations and the pursuit of our growth strategy;
- raising additional capital may cause dilution to our stockholders, restrict our operations or require us to relinquish rights to our technologies or product candidates;
- our business is dependent on our ability to advance our current and future product candidates through preclinical studies and clinical trials, obtain marketing approval and ultimately commercialize them. If we are unable to or experience significant delays in doing so, our business will be materially harmed;
- we are developing novel gene therapy product candidates, which makes it difficult to predict the time, cost and potential success of product candidate development;
- because gene therapy is novel and the regulatory landscape that governs any product candidates we may develop is rigorous, complex, uncertain and subject to change, we cannot predict with certainty the geographic areas in which we could obtain regulatory approval or the time and cost of obtaining regulatory approval, if we receive it at all, for any product candidates we may develop;

- preclinical studies and clinical trials are expensive, time-consuming, difficult to design and implement and involve an uncertain outcome. Further, we may encounter substantial delays in completing the development of our product candidates;
- the regulatory approval processes of the FDA, EMA and comparable foreign authorities are lengthy, time-consuming and inherently unpredictable. If we are not able to obtain required regulatory approval for our product candidates, our business will be substantially harmed;
- success in preclinical studies or earlier clinical trials may not be indicative of results in future clinical trials;
- interim “top-line” and preliminary results from our clinical trials that we announce, publish or present from time to time may change as more patient data become available and are subject to audit and verification procedures that could result in material changes in the final data;
- our preclinical studies and clinical trials may fail to demonstrate the safety and efficacy of our product candidates, or serious adverse or unacceptable side effects may be identified during the development of our product candidates, which could prevent or delay regulatory approval and commercialization, increase our costs or necessitate the abandonment or limitation of the development of some of our product candidates. We may also identify safety and efficacy concerns after the approval of a product candidate which can result in negative consequences to our business and results of operations;
- some of the diseases we initially seek to treat have low prevalence and it may be difficult to identify and enroll patients with these diseases. If we experience delays or difficulties in the enrollment and/or maintenance of patients in clinical trials, our receipt of necessary regulatory approvals could be delayed or prevented;
- we may seek Orphan Drug designation or Rare Pediatric Disease designation for some of our product candidates and we may be unsuccessful, or may be unable to maintain the benefits associated with Orphan Drug designation, including the potential for market exclusivity, for product candidates for which we obtain Orphan Drug designation;
- Fast Track, Breakthrough Therapy, or Regenerative Medicine Advanced Therapy designation that we may receive from the FDA may not actually lead to a faster development or regulatory review or approval process, and does not assure FDA approval of our product candidates;
- we have received Rare Pediatric Disease designation from the FDA for LX2006 for the treatment of FA and we may seek such designation for future product candidates. However, a marketing application for these product candidates, if approved, may not meet the eligibility criteria for a rare pediatric disease priority review voucher;
- we and our contract manufacturers are subject to significant regulation with respect to manufacturing our products. The third-party manufacturing facilities on which we rely, and any manufacturing facility that we may have in the future, may have limited capacity or fail to meet the applicable stringent regulatory requirements;
- gene therapies are novel, complex and difficult to manufacture. We could experience manufacturing problems that result in delays in the development or commercialization of our product candidates or otherwise harm our business;
- we depend on third-party suppliers for materials used in the manufacture of our product candidates, and the loss of these third-party suppliers or their inability to supply us with adequate materials could harm our business;
- even if any of our product candidates receive marketing approval, they may fail to achieve the degree of market acceptance by physicians, patients, third-party payors and others in the medical community necessary for commercial success;

- currently, we rely on our collaborations with Cornell University and UCSD to conduct research and development for many of our pipeline programs, including conducting preclinical and IND-enabling studies for portions of our near-term future pipeline. Failure or delay of Cornell University or UCSD to fulfill all or part of their respective obligations to us under our agreements, a breakdown in collaboration between the parties or a complete or partial loss of either of these relationships could materially harm our business;
- we intend to continue to rely on third parties to conduct a significant portion of our existing clinical trials and potential future clinical trials for product candidates, and those third parties may not perform satisfactorily, including failing to meet deadlines for the completion of such trials;
- if we are unable to obtain or protect intellectual property rights related to any of our product candidates, we may not be able to compete effectively in our market; and
- we are currently subject to a lawsuit claiming, among other things, that we misappropriated the confidential information and trade secrets of Rocket, and which seeks unspecified damages and asks the court to enjoin us from competing and working in the market for gene therapy treatments targeting cardiac diseases. In the future, we may be subject to additional claims that we and our employees, consultants, or independent contractors have wrongfully used or disclosed confidential information or trade secrets of third parties.

Implications of Being an Emerging Growth Company and a Smaller Reporting Company

We qualify as an “emerging growth company” as defined in the Jumpstart Our Business Startups Act of 2012, or the JOBS Act. For so long as we remain an emerging growth company, we may take advantage of relief from certain reporting requirements and other burdens that are otherwise applicable generally to public companies. These provisions include:

- reduced obligations with respect to financial data, including only being required to present two years of audited financial statements, in addition to any required unaudited interim financial statements with correspondingly reduced “Management’s Discussion and Analysis of Financial Condition and Results of Operations” disclosure;
- an exception from compliance with the auditor attestation requirements of Section 404 of the Sarbanes-Oxley Act of 2002, as amended;
- reduced disclosure about our executive compensation arrangements in our periodic reports, proxy statements and registration statements;
- exemptions from the requirements of holding non-binding advisory votes on executive compensation or golden parachute arrangements; and
- an exemption from compliance with the requirements of the Public Company Accounting Oversight Board regarding the communication of critical audit matters in the auditor’s report on financial statements.

We may take advantage of these provisions until we no longer qualify as an emerging growth company. We will cease to qualify as an emerging growth company on the date that is the earliest of: (i) the last day of our fiscal year following the fifth anniversary of the date of the completion of this offering, (ii) the last day of the fiscal year in which we have more than \$1.235 billion in total annual gross revenues, (iii) the date on which we are deemed to be a “large accelerated filer” under the rules of the U.S. Securities and Exchange Commission, which means the market value of our Common Stock that is held by non-affiliates exceeds \$700 million as of the prior June 30, or (iv) the date on which we have issued more than \$1.0 billion of non-convertible debt over the prior three-year period. We may choose to take advantage of some but not all of these reduced reporting burdens.

We have taken advantage of certain reduced reporting requirements in this prospectus. Accordingly, the information contained herein may be different than you might obtain from other public companies in which you hold equity interests.

In addition, under the JOBS Act, emerging growth companies can delay adopting new or revised accounting standards until such time as those standards apply to private companies. We have elected to take advantage of the extended transition period to comply with new or revised accounting standards and to adopt certain of the reduced disclosure requirements available to emerging growth companies. As a result of the accounting standards election, we will not be subject to the same implementation timing for new or revised accounting standards as other public companies that are not emerging growth companies, which may make comparison of our financials to those of other public companies more difficult. As a result of these elections, the information that we provide in this prospectus may be different than the information you may receive from other public companies in which you hold equity interests. In addition, it is possible that some investors will find our Common Stock less attractive as a result of these elections, which may result in a less active trading market for our Common Stock and higher volatility in our share price.

We are also a “smaller reporting company,” meaning that the market value of our shares held by non-affiliates plus the proposed aggregate amount of gross proceeds to us as a result of this offering is less than \$700 million and our annual revenue was less than \$100 million during the most recently completed fiscal year. We may continue to be a smaller reporting company after this offering if either (i) the market value of our shares held by non-affiliates is less than \$250 million or (ii) our annual revenue was less than \$100 million during the most recently completed fiscal year and the market value of our shares held by non-affiliates is less than \$700 million. If we are a smaller reporting company at the time we cease to be an emerging growth company, we may continue to rely on exemptions from certain disclosure requirements that are available to smaller reporting companies. Specifically, as a smaller reporting company, we may choose to present only the two most recent fiscal years of audited financial statements in our Annual Report on Form 10-K and, similar to emerging growth companies, smaller reporting companies have reduced disclosure obligations regarding executive compensation.

Corporate information

In February 2017, we were formed as a Delaware limited liability company under the name LEXEO Therapeutics, LLC. In November 2020, we converted into a Delaware corporation and were renamed Lexeo Therapeutics, Inc. Our principal executive offices are located at 345 Park Avenue South, Floor 6, New York, New York 10010, and our telephone number is (212) 547-9879. Our website address is www.lexeotx.com. The information contained on, or accessible through, our website is not incorporated by reference into this Annual Report. Our Annual Report on Form 10-K, Quarterly Reports on Form 10-Q, Current Reports on Form 8-K and amendments to reports filed pursuant to Sections 13(a) and 15(d) of the Securities Exchange Act of 1934, as amended, or the Exchange Act, are filed with the SEC. Such reports and other information filed by us with the SEC are available free of charge on our website at ir.lexeotx.com when such reports are available on the SEC’s website. The SEC maintains an internet site that contains reports, proxy and information statements and other information regarding issuers that file electronically with the SEC at www.sec.gov. The information contained on the websites referenced in prospectus is not incorporated by reference into this filing. Further, our references to website URLs are intended to be inactive textual references only.

On November 7, 2023, we completed our initial public offering, or IPO, pursuant to which we issued and sold 9,090,910 shares of our Common Stock. Subsequently, we issued and sold 1,048,746 additional shares in connection with the partial exercise by the underwriters of their 30-day option to purchase additional shares of Common Stock at a public offering price of \$11.00 per share. The total gross proceeds from the IPO and the subsequent partial exercise of the underwriters’ 30-day option to purchase additional shares of Common Stock

were approximately \$111.5 million and the total net proceeds were approximately \$100.3 million, after deducting underwriting discounts and commissions and other offering expenses. In addition, on March 13, 2024, we issued and sold 6,278,905 shares of our Common Stock at a price per share of \$15.13 pursuant to the Private Placement. The gross proceeds from the Private Placement were approximately \$95 million, and the total net proceeds were approximately \$88.9 million, after deducting commissions and offering expenses.

Channels of Distribution

We announce material information to the public through filings with the SEC, the investor relations page on our website, press releases, public conference calls, and webcasts in order to achieve broad, non-exclusionary distribution of information to the public and for complying with our disclosure obligations under Regulation FD. We encourage investors, the media, and others to follow the channels listed above and to review the information disclosed through such channels. Any updates to the list of disclosure channels through which we will announce information will be posted on the investor relations page on our website.

THE OFFERING

Resale of Common Stock

Shares of Common Stock offered by the Selling Stockholders hereunder 6,974,248 shares

Use of Proceeds

We will not receive any proceeds from the sale of our Common Stock offered by the Selling Stockholders under this prospectus (the “Securities”). See the section of this prospectus titled “*Use of Proceeds*” appearing elsewhere in this prospectus for more information.

Risk Factors

See the section titled “*Risk Factors*” beginning on page 13 of this prospectus and other information included in this prospectus for a discussion of factors that you should consider carefully before deciding to invest in our Common Stock.

Nasdaq Global Market Symbol

“LXEO”

Lock-Up Restrictions

Of the 6,974,248 shares of Common Stock that may be offered or sold by Selling Stockholders identified in this prospectus, 695,343 of those shares are subject to lock-up restrictions until April 30, 2024, pursuant to contractual agreements further described in the section titled “*Certain Relationships and Related Person Transactions*” appearing elsewhere in this prospectus.

Unless otherwise noted, the number of our shares of Common Stock outstanding is based on 32,948,332 shares of Common Stock outstanding as of March 27, 2024, and excludes:

- 3,277,349 shares of our Common Stock issuable upon the exercise of outstanding options, with a weighted-average exercise price of \$10.42 per share;
- 224,945 shares of our Common Stock issuable upon the vesting of restricted stock units;
- 2,539,744 shares of our Common Stock reserved for future issuance under our 2023 Equity Incentive Plan (the “2023 Plan”); and
- 505,284 shares of our Common Stock reserved for future issuance under our 2023 Employee Stock Purchase Plan (the “2023 ESPP”).

Unless otherwise noted, the information in this prospectus assumes no exercise of outstanding options subsequent to March 27, 2024.

RISK FACTORS

Investing in our Common Stock involves a high degree of risk. Before making an investment decision, you should consider carefully the risks and uncertainties described below, together with all of the other information in this prospectus, including the section titled "Management's Discussion and Analysis of Financial Condition and Results of Operations" beginning on page 86 of this prospectus and our consolidated financial statements and related notes thereto included elsewhere in this prospectus. Our business, operating results, financial condition or prospects could also be harmed by risks and uncertainties not currently known to us or that we currently do not believe are material. If any of the risks actually occur, our business, operating results, financial condition and prospects could be adversely affected. In that event, the market price of our Common Stock could decline, and you could lose part or all of your investment.

Risk Factors Summary

Investing in our Common Stock involves a high degree of risk because our business is subject to numerous risks and uncertainties, as fully described below. The principal factors and uncertainties that make investing in our Common Stock risky include, among others:

- we have incurred significant losses since our inception, and we expect to incur significant net losses for the foreseeable future and may not be able to achieve or sustain revenue or profitability in the future;
- we have a limited operating history, have not completed any clinical trials, and have no products approved for commercial sale;
- if we are unable to raise capital when needed, we could be forced to curtail our planned operations and the pursuit of our growth strategy;
- raising additional capital may cause dilution to our stockholders, restrict our operations or require us to relinquish rights to our technologies or product candidates;
- our business is dependent on our ability to advance our current and future product candidates through preclinical studies and clinical trials, obtain marketing approval and ultimately commercialize them. If we are unable to or experience significant delays in doing so, our business will be materially harmed;
- we are developing novel gene therapy product candidates, which makes it difficult to predict the time, cost and potential success of product candidate development;
- because gene therapy is novel and the regulatory landscape that governs any product candidates we may develop is rigorous, complex, uncertain and subject to change, we cannot predict with certainty the geographic areas in which we could obtain regulatory approval or the time and cost of obtaining regulatory approval, if we receive it at all, for any product candidates we may develop;
- preclinical studies and clinical trials are expensive, time-consuming, difficult to design and implement and involve an uncertain outcome. Further, we may encounter substantial delays in completing the development of our product candidates;
- the regulatory approval processes of the FDA, EMA and comparable foreign authorities are lengthy, time-consuming and inherently unpredictable. If we are not able to obtain required regulatory approval for our product candidates, our business will be substantially harmed;
- success in preclinical studies or earlier clinical trials may not be indicative of results in future clinical trials;
- interim "top-line" and preliminary results from our clinical trials that we announce, publish or present from time to time may change as more patient data become available and are subject to audit and verification procedures that could result in material changes in the final data;
- our preclinical studies and clinical trials may fail to demonstrate the safety and efficacy of our product candidates, or serious adverse or unacceptable side effects may be identified during the development of

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our product candidates, which could prevent or delay regulatory approval and commercialization, increase our costs or necessitate the abandonment or limitation of the development of some of our product candidates. We may also identify safety and efficacy concerns after the approval of a product candidate which can result in negative consequences to our business and results of operations;

- some of the diseases we initially seek to treat have low prevalence and it may be difficult to identify and enroll patients with these diseases. If we experience delays or difficulties in the enrollment and/or maintenance of patients in clinical trials, our receipt of necessary regulatory approvals could be delayed or prevented;
- we may seek Orphan Drug designation or Rare Pediatric Disease designation for some of our product candidates and we may be unsuccessful, or may be unable to maintain the benefits associated with Orphan Drug designation, including the potential for market exclusivity, for product candidates for which we obtain Orphan Drug designation;
- Fast Track, Breakthrough Therapy, or Regenerative Medicine Advanced Therapy designation that we may receive from the FDA may not actually lead to a faster development or regulatory review or approval process, and does not assure FDA approval of our product candidates;
- we have received Rare Pediatric Disease designation from the FDA for LX2006 for the treatment of FA and we may seek such designation for future product candidates. However, a marketing application for these product candidates, if approved, may not meet the eligibility criteria for a rare pediatric disease priority review voucher;
- we and our contract manufacturers are subject to significant regulation with respect to manufacturing our products. The third-party manufacturing facilities on which we rely, and any manufacturing facility that we may have in the future, may have limited capacity or fail to meet the applicable stringent regulatory requirements;
- gene therapies are novel, complex and difficult to manufacture. We could experience manufacturing problems that result in delays in the development or commercialization of our product candidates or otherwise harm our business;
- we depend on third-party suppliers for materials used in the manufacture of our product candidates, and the loss of these third-party suppliers or their inability to supply us with adequate materials could harm our business;
- even if any of our product candidates receive marketing approval, they may fail to achieve the degree of market acceptance by physicians, patients, third-party payors and others in the medical community necessary for commercial success;
- currently, we rely on our collaborations with Cornell University and UCSD to conduct research and development for many of our pipeline programs, including conducting preclinical and IND-enabling studies for portions of our near-term future pipeline. Failure or delay of Cornell University or UCSD to fulfill all or part of their respective obligations to us under our agreements, a breakdown in collaboration between the parties or a complete or partial loss of either of these relationships could materially harm our business;
- we intend to continue to rely on third parties to conduct a significant portion of our existing clinical trials and potential future clinical trials for product candidates, and those third parties may not perform satisfactorily, including failing to meet deadlines for the completion of such trials;
- if we are unable to obtain or protect intellectual property rights related to any of our product candidates, we may not be able to compete effectively in our market; and
- we are currently subject to a lawsuit claiming, among other things, that we misappropriated the confidential information and trade secrets of Rocket, and which seeks unspecified damages and asks the court to enjoin us from competing and working in the market for gene therapy treatments targeting

cardiac diseases. In the future, we may be subject to additional claims that we and our employees, consultants, or independent contractors have wrongfully used or disclosed confidential information or trade secrets of third parties.

Risks related to our financial position and capital needs

We have incurred significant losses since our inception. We expect to incur losses over the next several years and may never achieve or maintain profitability.

Since our inception, we have incurred significant net losses, and we expect to continue to incur significant expenses and operating losses for the foreseeable future. For the fiscal years ended December 31, 2023 and 2022, we incurred net losses of \$66.4 million and \$59.3 million, respectively, and we had an accumulated deficit of \$181.8 million as of December 31, 2023. We have primarily financed our operations with approximately of \$100.3 million of net proceeds raised in our IPO and the subsequent partial exercise of the underwriters' 30-day option to purchase additional shares of Common Stock, as well as totals of approximately \$88.9 million, \$185.0 million and \$3.9 million of net proceeds from the Private Placement, sales of our convertible equity securities, and a convertible SAFE Note, respectively. We have no products approved for commercialization and have never generated any revenue from product sales.

We are still in the early clinical stages of development of our lead product candidates. We expect to continue to incur significant expenses and operating losses over the next several years. Our operating expenses and net losses may fluctuate significantly from quarter to quarter and year to year. We anticipate that our expenses will increase substantially as we:

- continue to advance the preclinical and clinical development of our product candidates and discovery programs;
- initiate and complete additional clinical trials of our current and future product candidates;
- seek regulatory approval for any product candidates that successfully complete clinical trials;
- continue to develop our gene therapy product candidate pipeline;
- scale up our clinical and regulatory capabilities;
- work with our third party manufacturing partners to produce material in accordance with cGMP for clinical trials or potential commercial sales;
- establish, either alone or with a third party, a commercialization infrastructure and scale up manufacturing and distribution capabilities to commercialize any product candidates for which we may obtain regulatory approval;
- adapt our regulatory compliance efforts to incorporate requirements applicable to marketed products;
- maintain, expand and protect our intellectual property portfolio and patent claims;
- hire additional clinical, quality control, regulatory, manufacturing, scientific and administrative personnel;
- add operational, financial and management information systems and personnel, including personnel to support our product development and planned future commercialization efforts; and
- incur additional legal, accounting and other expenses in operating as a public company.

To date, we have not generated any revenue from the commercialization of our product candidates. To become and remain profitable, we must succeed in developing and eventually commercializing product candidates that generate significant revenue. This will require us to be successful in a range of challenging activities, including completing preclinical testing and clinical trials of our product candidates, obtaining

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regulatory approval, and manufacturing, marketing and selling any product candidates for which we may obtain regulatory approval, as well as discovering and developing additional product candidates. We are only in the preliminary stages of most of these activities and all of our product candidates are in early clinical trials or preclinical development. We may never succeed in these activities and, even if we do, may never generate any revenue or revenue that is significant enough to achieve profitability.

Even if we achieve profitability, we may not be able to sustain or increase profitability on a quarterly or annual basis. Our failure to become and remain profitable would depress the value of our company and could impair our ability to raise capital, expand our business, maintain our development efforts, obtain product approvals, diversify our offerings or continue our operations. A decline in the value of our company could also cause you to lose all or part of your investment.

We have a limited operating history and no history of commercializing products, which may make it difficult for an investor to evaluate the success of our business to date and to assess our future viability.

We are a clinical stage genetic medicine company with a limited operating history. We commenced substantive business operations in 2020, and our operations to date have been largely focused on organizing and staffing our company, business planning, raising capital and entering into collaboration and license agreements for conducting preclinical and clinical research and development activities for our product candidates and gene therapy pipeline. To date, we have not yet demonstrated our ability to successfully complete internally sponsored clinical trials, complete pivotal clinical trials, manufacture a product on a commercial scale or arrange for a third party to do so on our behalf, or conduct sales and marketing activities necessary for successful commercialization. Consequently, any predictions you make about our future success or viability may not be as accurate as they could be if we had a longer operating history or a history of successfully developing and commercializing products.

We will need substantial additional funding to meet our financial obligations and to pursue our business objectives. If we are unable to raise capital when needed, we could be forced to curtail our planned operations and the pursuit of our growth strategy.

We will require substantial future capital in order to complete planned and future clinical development for our lead product candidates, preclinical development for our other product candidates, and potential commercialization of these product candidates, if any are approved. We expect our spending levels to significantly increase in connection with our planned clinical trials of our lead product candidates. In addition, if we obtain marketing approval for any of our product candidates, we expect to incur significant expenses related to product sales, medical affairs, marketing, manufacturing and distribution. We also expect to incur additional costs associated with operating as a public company. Accordingly, we will need to obtain substantial additional funding in connection with our continuing operations. If we are unable to raise capital when needed or on acceptable terms, we would be forced to delay, reduce or eliminate certain of our clinical trials, our research and development programs or other operations.

As of December 31, 2023, we had cash and cash equivalents of \$121.5 million. Following the approximately \$88.9 million of net proceeds expected to be received upon the anticipated closing of the Private Placement in March 2024, we believe that our cash and cash equivalents will be sufficient to fund our operating expenses and capital requirements into 2027. This estimate is based on assumptions that may prove to be wrong, and we could use our available capital resources sooner than we expect. Changes may occur beyond our control that would cause us to consume our available capital before that time, including changes in and progress of our development activities, acquisitions of additional product candidates, and changes in regulation. Our future capital requirements will depend on many factors, including:

- the costs of and investment in ongoing and future development of our gene therapy product candidates;
- the scope, progress, costs and results of discovery, preclinical development, laboratory testing and clinical trials for our product candidates;

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- the extent to which we develop, in-license or acquire other product candidates and technologies in our product candidate pipeline;
- the costs and timing of process development and manufacturing scale-up activities associated with our product candidates and other programs as we advance them through preclinical and clinical development;
- the number of, and development requirements for, product candidates that we may pursue;
- the costs, timing and outcome of regulatory review of our product candidates;
- our headcount growth and associated costs as we expand our research and development capabilities and establish a commercial infrastructure;
- the costs of establishing and maintaining commercial-scale cGMP manufacturing capabilities, either internally or with third parties;
- the costs and timing of future commercialization activities, including product manufacturing, marketing, sales and distribution, for any of our product candidates for which we receive marketing approval;
- the costs and timing of preparing, filing and prosecuting patent applications, maintaining and enforcing our intellectual property rights and defending any intellectual property-related claims;
- the revenue, if any, received from commercial sales of our product candidates for which we receive marketing approval;
- our ability to establish and maintain additional collaborations on favorable terms, if at all;
- the success of any collaborations that we may establish and our license agreements;
- the outcome of legal proceedings involving us;
- the achievement of milestones or occurrence of other developments that trigger payments under our collaboration agreement or any additional collaboration agreements we may enter into; and
- the costs of operating as a public company.

We will require additional capital to achieve our business objectives. While the long-term economic impact of either the COVID-19 pandemic or the ongoing geopolitical conflicts in Ukraine and Israel is difficult to assess or predict, each of these events has caused significant disruptions to the global financial markets and contributed to a general global economic slowdown. Furthermore, inflation rates, particularly in the United States, have increased recently to levels not seen in decades. Increased inflation may result in increased operating costs and may affect our operating budgets, specifically with respect to increased labor costs and associated difficulties in recruiting qualified personnel. In addition, the U.S. Federal Reserve has raised, and may further raise, interest rates in response to concerns about inflation. Increases in interest rates, especially if coupled with reduced government spending and volatility in financial markets, may further increase economic uncertainty and heighten these risks. If the disruptions and slowdown deepen or persist, we may not be able to access additional capital on favorable terms, or at all, which could in the future negatively affect our financial condition and we could be forced to curtail our planned operations and the pursuit of our growth strategy.

Raising additional capital may cause dilution to our stockholders, restrict our operations or require us to relinquish rights to our technologies or product candidates.

Until such time, if ever, as we can generate substantial revenue, we may finance our cash needs through a combination of equity offerings, government or private party grants, debt financings and license and collaboration agreements. We do not currently have any committed external source of funds. To the extent that we raise additional capital through the sale of equity or convertible debt securities, your ownership interest will

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be diluted, and the terms of these securities may include liquidation or other preferences that adversely affect your rights as a common stockholder. Debt financing and preferred equity financing, if available, may involve agreements that include covenants limiting or restricting our ability to take specific actions, such as incurring additional debt, making capital expenditures or declaring dividends.

If we raise additional funds through collaborations, strategic alliances or marketing, distribution or licensing arrangements with third parties, we may be required to relinquish valuable rights to our technologies, future revenue streams or product candidates, grant licenses on terms that may not be favorable to us or commit to future payment streams. If we are unable to raise additional funds through equity or debt financings when needed, we may be required to delay, limit, reduce or terminate our product development or future commercialization efforts or grant rights to develop and market product candidates that we would otherwise prefer to develop and market ourselves.

Risks related to the development of our product candidates

Our business is dependent on our ability to advance our current and future product candidates through preclinical studies and clinical trials, obtain marketing approval and ultimately commercialize them. If we are unable to or experience significant delays in doing so, our business will be materially harmed.

We have invested a significant portion of our time and financial resources in the development of our product candidates and technology platforms. Our business is dependent on our ability to successfully complete preclinical and clinical development of, obtain regulatory approval for, and, if approved, successfully commercialize LX2006, LX1001, LX2020 and any other product candidates in a timely manner.

Each of our product candidates and programs will require additional preclinical and/or clinical development, regulatory approval and significant marketing efforts, and we will be required to obtain manufacturing supply and expertise and to build a commercial organization or successfully outsource commercialization before we generate any revenue from product sales. We do not have any products that are approved for commercial sale, and we may never be able to develop or commercialize marketable products.

Our ability to generate revenue from our product candidates, which we do not expect to occur for several years, if ever, will depend heavily on the successful development, regulatory approval and eventual commercialization of our product candidates. The success of our lead product candidates, or any other product candidates that we develop or otherwise may acquire will depend on several factors, including:

- timely and successful completion of preclinical studies, including toxicology studies, biodistribution studies and minimally efficacious dose studies in animals, where applicable, under GLPs;
- the availability or development of suitable animal disease models for nonclinical studies to enable us to proceed into clinical development or support the submission of a marketing application;
- effective IND applications from the FDA or comparable foreign applications that allow commencement of our planned clinical trials or future clinical trials for our product candidates;
- sufficiency of our financial and other resources to complete the necessary preclinical studies and clinical trials;
- successful enrollment and completion of clinical trials, including under cGCPs;
- establishment of our own manufacturing capabilities and/or arrangements with third-party manufacturers for our commercial manufacturing processes for any of our product candidates that receive regulatory approval;
- receipt of timely marketing approvals from applicable regulatory authorities;
- launch of commercial sales of products, if approved, whether alone or in collaboration with others;

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- acceptance of the benefits and use of our products, including method of administration, if and when approved, by patients, the medical community and third-party payors, for their approved indications;
- the prevalence and severity of adverse events experienced with any of our product candidates;
- the availability, perceived advantages, cost, safety and efficacy of alternative therapies for any diseases for which we are developing our product candidates;
- our ability to produce our product candidates on a commercial scale;
- attainment and maintenance of patent, trademark and trade secret protection and regulatory exclusivity for our product candidates and otherwise protecting our rights in our intellectual property portfolio;
- maintenance of compliance with regulatory requirements such as cGMPs;
- attainment and maintenance of third-party coverage and adequate reimbursement for our product candidates and patients' willingness to pay out-of-pocket in the absence of such coverage and adequate reimbursement; and
- maintenance of a continued acceptable safety, tolerability and efficacy profile of our products following approval.

If we are not successful with respect to one or more of these factors in a timely manner or at all, we could experience significant delays or an inability to successfully commercialize the product candidates we develop, which would materially harm our business. If we do not receive marketing approvals for any product candidate we develop, we may not be able to continue our operations.

We are developing novel gene therapy product candidates, which makes it difficult to predict the time, cost and potential success of product candidate development.

Our future success depends on the successful development of a novel therapeutic approach. To date, very few products that utilize gene transfer have been approved in the United States or Europe. There have been a limited number of clinical trials using AAVrh10. Although gene therapies have been studied in human clinical trials for over 30 years, only a limited number of AAV-based gene therapy products have been approved by the FDA.

We cannot be certain that our AAVrh10-based gene therapy product candidates will successfully complete clinical trials or that any future product candidates utilizing this or other vector constructs will successfully complete preclinical studies or clinical trials. We may not be successful in developing product candidates that avoid triggering toxicities or other side effects in preclinical studies or clinical trials. Our intravenous, intracisternal and intrathecal routes of administration may cause unforeseen side effects or present other challenges. Any such results could impact our ability to develop a product candidate, including our ability to enroll patients in our clinical trials. As a result of these factors, it is more difficult for us to predict the time and cost of product candidate development, and we cannot predict whether the application of our approach to gene therapy, or any similar or competitive programs, will result in the identification, development, and regulatory approval of any product candidate, or that other gene therapy programs will not be considered better or more favorable. There can be no assurance that any development problems we experience in the future related to our current gene therapy product candidates or any of our research programs will not cause significant delays or unanticipated costs, or that such development problems can be solved. We may also experience delays and challenges in achieving sustainable, reproducible, and scalable production. Any of these factors may prevent us from completing our preclinical studies or clinical trials or commercializing any product candidates we may develop on a timely or profitable basis, if at all.

Because gene therapy is novel and the regulatory landscape that governs any product candidates we may develop is rigorous, complex, uncertain and subject to change, we cannot predict with certainty the geographic areas in which we could obtain regulatory approval or the time and cost of obtaining regulatory approval, if we receive it at all, for any product candidates we may develop.

The regulatory requirements that will govern any novel gene therapy product candidates we develop are not entirely clear and are subject to change. The novel nature of our capsids makes it difficult to determine how long it will take or how much it will cost to obtain regulatory approvals for our product candidates in the United States, the European Union or other jurisdictions. Within the broader genetic medicine field, very few gene therapy products have received marketing authorization from the FDA or the European Medicines Agency, or EMA. Even with respect to gene therapies, the regulatory landscape is still developing. Regulatory requirements governing gene therapy products have changed frequently and will likely continue to change in the future, including with respect to those responsible for regulation of existing gene therapy products. For example, in 2016, the FDA established the Office of Tissues and Advanced Therapies, or OTAT, within the Center for Biologics Evaluation and Research, or CBER, to consolidate the review of gene therapy and related products, and to advise the CBER on its review. In September 2022, the FDA announced retitling of OTAT to the Office of Therapeutic Products, or OTP, and elevation of OTP to a “Super Office” to meet its growing cell and gene therapy workload.

Our product candidates will need to meet safety and efficacy standards applicable to any new biologic being pursued for a given disease under the regulatory framework administered by the FDA. Although the FDA decides whether individual gene therapy protocols may proceed, the review process and determinations of other reviewing bodies, including IRBs, can impede or delay the initiation of a clinical trial.

The same applies in the EU. The EMA’s Committee for Advanced Therapies, or CAT, is responsible for assessing the quality, safety and efficacy of advanced-therapy medicinal products. Advanced-therapy medicinal products include gene therapy medicines, somatic-cell therapy medicines and tissue-engineered medicines. The role of the CAT is to prepare a draft opinion on an application for marketing authorization for a gene therapy medicinal candidate that is submitted to the EMA. In the EU, the development and evaluation of a gene therapy product must be considered in the context of the relevant EU guidelines. The EMA may issue new guidelines concerning the development and marketing authorization for gene therapy products and require that we comply with these new guidelines. This could mean that any gene therapy product candidate we may develop in the future could be required to comply with additional and/or more stringent gene therapy guidelines in the EU.

Adverse developments in preclinical studies or clinical trials conducted by others in the field of gene therapy and gene regulation products may cause the FDA, the EMA and other regulatory bodies to revise the requirements for approval of any product candidates we may develop or limit the use of products utilizing gene regulation technologies, either of which could harm our business. In addition, the clinical trial requirements of the FDA, the EMA and other regulatory authorities and the criteria these regulators use to determine the safety and efficacy of a product candidate vary substantially according to the type, complexity, novelty, and intended use and market of the potential products. The regulatory approval process for product candidates such as ours can be more expensive and take longer than for other, better known, or more extensively studied pharmaceutical or other product candidates. Further, as we are developing novel potential treatments for diseases in which, in some cases, there is little clinical experience with potential new endpoints and methodologies, there is heightened risk that the FDA, the EMA or other regulatory bodies may not consider the clinical trial endpoints to provide clinically meaningful results, and the resulting clinical data and results may be more difficult to analyze. In addition, we may not be able to identify or develop appropriate animal disease models to enable or support planned clinical development. Any natural history studies that we may rely upon in our clinical development may not be accepted by the FDA, EMA or other regulatory authorities. Regulatory agencies administering existing or future regulations or legislation may not allow production and marketing of products utilizing gene regulation technology in a timely manner or under technically or commercially feasible conditions. In addition, regulatory action or private litigation could result in expenses, delays, or other impediments to our research programs or the

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commercialization of resulting products. Further, approvals by one regulatory agency may not be indicative of what other regulatory agencies may require for approval.

The regulatory review committees and advisory groups described above and any new guidelines they promulgate may lengthen the regulatory review process, require us to perform additional preclinical studies or clinical trials, increase our development costs, lead to changes in regulatory positions and interpretations, delay or prevent approval and commercialization of these treatment product candidates, or lead to significant post-approval limitations or restrictions. As we advance our research programs and develop future product candidates, we will be required to consult with these regulatory and advisory groups and to comply with applicable guidelines. If we fail to do so, we may be required to delay or discontinue development of any product candidates we identify and develop. These additional processes may result in a review and approval process that is longer than we otherwise would have expected. Delays as a result of an increased or lengthier regulatory approval process or further restrictions on the development of our product candidates can be costly and could negatively impact our ability to complete clinical trials and commercialize our current and future product candidates in a timely manner, if at all.

Preclinical studies and clinical trials are expensive, time-consuming, difficult to design and implement and involve an uncertain outcome. Further, we may encounter substantial delays in completing the development of our product candidates.

All of our product candidates are in preclinical or early clinical development, and the risk of failure is high. The preclinical studies, clinical trials and manufacturing of our product candidates are, and the manufacturing and marketing of our products, if approved, will be, subject to extensive and rigorous review and regulation by numerous government authorities in the United States and in other countries where we may test and market our product candidates. Before obtaining regulatory approvals for the commercial sale of any of our product candidates, we must demonstrate through lengthy, complex and expensive preclinical testing and clinical trials that our product candidates are both safe and effective for use in each target disease. In particular, because our product candidates are subject to regulation as biologics, we will need to demonstrate that they are sufficiently safe and of sufficient purity and potency for use in their target diseases. Each product candidate must demonstrate an adequate risk-versus-benefit profile in its intended patient population and for its intended use.

Clinical testing is expensive, can take many years to complete and is subject to uncertainty. We cannot guarantee that any clinical trials will be initiated on schedule, conducted as planned or completed on schedule, if at all. To date, we are sponsoring clinical trials of LX1001, LX2006 and LX2020, but we have not successfully completed any clinical trial that we have internally sponsored. Failure can occur at any time during the clinical trial process. Even if our ongoing and future clinical trials are completed as planned, we cannot be certain that their results will support the safety and effectiveness of our product candidates for their targeted diseases or support continued clinical development of such product candidates. Our future clinical trial results may not be successful.

In addition, even if such trials are successfully completed, we cannot guarantee that the FDA or foreign regulatory authorities will interpret the results as we do, and more trials could be required before we submit our product candidates for approval. This is particularly true for clinical trials in rare diseases, where the small patient populations make it difficult or impossible to conduct two traditional, adequate and well-controlled trials, and therefore the FDA or comparable foreign regulatory authorities are often required to exercise flexibility in approving therapies for such diseases. Moreover, results acceptable to support approval in one jurisdiction may be deemed inadequate by another regulatory authority to support regulatory approval in that other jurisdiction. To the extent that the results of the trials are not satisfactory to the FDA or foreign regulatory authorities for support of a marketing application, we may be required to expend significant resources, which may not be available to us, to conduct additional trials in support of potential approval of our product candidates.

We may experience delays in initiating and conducting clinical trials of our lead product candidates and we do not know whether our clinical trials will begin on time, need to be redesigned, recruit and enroll patients on

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time or be completed on schedule, or at all. Events that may prevent successful or timely completion of clinical development include:

- inability to generate sufficient preclinical, toxicology, or other *in vivo* or *in vitro* data to support the initiation of clinical trials;
- delays in sufficiently developing, characterizing or controlling a manufacturing process suitable for advanced clinical trials;
- delays in sourcing or qualifying ancillaries required for administration of our clinical drug product (such as vials, stoppers, or tubing);
- delays in developing suitable assays for screening patients for eligibility for trials with respect to certain product candidates;
- delays in reaching agreement with the FDA, EMA or other regulatory authorities as to the design or implementation of our clinical trials;
- failure to obtain regulatory approval to commence a clinical trial;
- failure to reach an agreement on acceptable terms with clinical trial sites or prospective CROs, the terms of which can be subject to extensive negotiation and may vary significantly among different clinical trial sites;
- inability to obtain IRB approval for each clinical trial site;
- inability to recruit suitable patients to participate in a clinical trial in a timely manner;
- failure to have patients complete a clinical trial or return for post-treatment follow-up;
- deviations by clinical trial sites, CROs or other third parties from trial protocol;
- failure to perform our planned clinical trials in accordance with the FDA's cGCP requirements, or applicable regulatory guidelines in other countries;
- inability to address patient-safety concerns that arise during the course of a trial, including occurrence of adverse events associated with the product candidate that are viewed to outweigh its potential benefits;
- failure to initiate a sufficient number of clinical trial sites; or
- delays in manufacturing sufficient quantities of a product candidate for use in clinical trials.

We may experience numerous unforeseen events during, or as a result of, clinical trials that could delay or prevent our ability to receive marketing approval or commercialize our product candidates or significantly increase the cost of such trials, including:

- we may experience changes in regulatory requirements or guidance, or receive feedback from regulatory authorities that requires us to modify the design of our clinical trials;
- clinical trials of our product candidates may produce negative or inconclusive results, and we may decide, or regulators may require us, to conduct additional clinical trials or halt development programs;
- the number of patients required for clinical trials of our product candidates may be larger than we anticipate, enrollment in these clinical trials may be slower than we anticipate, or participants may drop out of these clinical trials at a higher rate than we anticipate;
- our third-party contractors may fail to comply with regulatory requirements or meet their contractual obligations to us in a timely manner, or at all;
- we, our investigators or regulators may suspend or terminate clinical trials of our product candidates for various reasons, including non-compliance with regulatory requirements, a finding that our product candidates have undesirable side effects or other unexpected characteristics, or a finding that the participants are being exposed to unacceptable health risks;

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- the cost of clinical trials of our product candidates may be greater than we anticipate, and we may not have funds to cover the costs;
- the supply or quality of our product candidates or other materials necessary to conduct clinical trials of our product candidates may be insufficient or inadequate;
- regulators may revise the requirements for approving our product candidates, or such requirements may not be as we anticipate; and
- any future collaborators that conduct clinical trials may face any of the above issues and may conduct clinical trials in ways they view as advantageous to them but that are suboptimal for us.

If we are required to conduct additional clinical trials or other testing of our product candidates beyond those that we currently contemplate, if we are unable to successfully initiate or complete clinical trials of our product candidates or other testing, if the results of these trials or tests are not positive or are only modestly positive or if there are safety concerns, we may:

- incur unplanned costs;
- be delayed in obtaining marketing approval for our product candidates or not obtain marketing approval at all;
- obtain marketing approval in some countries and not in others;
- obtain marketing approval for diseases or patient populations that are not as broad as intended or desired;
- obtain marketing approval with labeling that includes significant use or distribution restrictions or safety warnings, including boxed warnings or REMS;
- be subject to additional post-marketing testing requirements; or
- have the product removed from the market after obtaining marketing approval.

We could encounter delays if a clinical trial is suspended or terminated by us, by the IRBs of the institutions in which such trials are being conducted, by the Data Safety Monitoring Board for such trial or by the FDA, EMA or other regulatory authorities. Such authorities may impose such a suspension or termination due to a number of factors, including failure to conduct the clinical trial in accordance with regulatory requirements or our clinical protocols, inspection of the clinical trial operations or trial site by the FDA, EMA or other regulatory authorities resulting in the imposition of a clinical hold, unforeseen safety issues or adverse side effects, failure to demonstrate a benefit from using a drug, changes in governmental regulations or administrative actions or lack of adequate funding to continue the clinical trial.

All of our product candidates will require extensive clinical testing before we are prepared to submit a BLA or marketing authorization application, or MAA, for regulatory approval. We cannot predict with any certainty if or when we might complete the clinical development for our product candidates and submit a BLA or MAA for regulatory approval of any of our product candidates or whether any such BLA or MAA will be approved. We may also seek feedback from the FDA, EMA or other regulatory authorities on our clinical development program, and the FDA, EMA or such regulatory authorities may not provide such feedback on a timely basis, or such feedback may not be favorable, which could further delay our development programs.

We cannot predict with any certainty whether or when we might complete a given clinical trial. If we experience delays in the commencement or completion of our clinical trials, or if we terminate a clinical trial prior to completion, the commercial prospects of our product candidates could be harmed, and our ability to generate revenues from our product candidates may be delayed or lost. In addition, any delays in our clinical trials could increase our costs, slow down the development and approval process and jeopardize our ability to commence product sales and generate revenues. Any of these occurrences may harm our business, financial

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condition and results of operations. In addition, many of the factors that cause, or lead to, a delay in the commencement or completion of clinical trials may also ultimately lead to the denial of regulatory approval of our product candidates.

The regulatory approval processes of the FDA, EMA and comparable foreign authorities are lengthy, time-consuming and inherently unpredictable. If we are not able to obtain required regulatory approval for our product candidates, our business will be substantially harmed.

The time required to obtain approval or other marketing authorizations by the FDA, EMA and comparable foreign authorities is unpredictable, and it typically takes many years following the commencement of clinical trials and depends upon numerous factors, including the substantial discretion of the regulatory authorities. In addition, approval policies, regulations, and the type and amount of clinical data necessary to gain approval may change during the course of a product candidate's clinical development and may vary among jurisdictions. We have not obtained regulatory approval for any product candidate, and it is possible that we may never obtain regulatory approval for any product candidates we may seek to develop in the future. Neither we nor any collaborator is permitted to market any of our biologic product candidates in the United States until we receive regulatory approval of a BLA from the FDA, and we cannot market any of our product candidates in the European Union until we receive approval for an MAA from the EMA, or other required regulatory approval in other countries.

Prior to obtaining approval to commercialize any product candidate in the United States or abroad, we must demonstrate with substantial evidence from well-controlled clinical trials, and to the satisfaction of the FDA or foreign regulatory agencies, that such product candidates are safe, effective and of sufficient purity for their intended uses. Results from preclinical studies and clinical trials can be interpreted in different ways. Even if we believe the preclinical or clinical data for our product candidates meet regulatory standards, such data may not be sufficient to support approval by the FDA and other regulatory authorities. The FDA may also require us to conduct additional preclinical studies or clinical trials for our product candidates either prior to or after approval, or it may object to elements of our clinical development programs, or require changes to our manufacturing approaches.

Of the large number of products in development, only a small percentage successfully complete the FDA or foreign regulatory approval processes and are commercialized. The lengthy approval and marketing authorization process as well as the unpredictability of future clinical trial results may result in our failing to obtain regulatory approval and marketing authorization to market our product candidates, which would significantly harm our business, financial condition, results of operations and prospects.

We have invested a significant portion of our time and financial resources in the development of our product candidates and technology platforms. Our business is dependent on our ability to successfully complete preclinical and clinical development of, obtain regulatory approval for, and, if approved, successfully commercialize LX2006, LX2020, LX1001 and our other product candidates in a timely manner.

Even if we eventually complete clinical testing and receive approval of a BLA or foreign marketing application for any of our product candidates, the FDA, EMA or the applicable foreign regulatory agency may grant approval or other marketing authorization contingent on the performance of costly additional clinical trials, including post-marketing clinical trials. The FDA, EMA or the applicable foreign regulatory agency also may approve or authorize for marketing a product candidate for a more limited disease or patient population than we originally request, and the FDA, EMA or applicable foreign regulatory agency may not approve or authorize the labeling that we believe is necessary or desirable for the successful commercialization of a product candidate. Any delay in obtaining, or inability to obtain, applicable regulatory approval or other marketing authorization would delay or prevent commercialization of that product candidate and would materially adversely impact our business and prospects.

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In addition, the FDA, EMA and other regulatory authorities may change their policies, issue additional regulations or revise existing regulations, or take other actions, which may prevent or delay approval of our future products under development on a timely basis. Such policy or regulatory changes could impose additional requirements upon us that could delay our ability to obtain approvals, increase the costs of compliance or restrict our ability to maintain any marketing authorizations we may have obtained. In addition, if the Supreme Court reverses or curtails the *Chevron* doctrine, which gives deference to regulatory agencies in litigation against FDA and other agencies, more companies may bring lawsuits against FDA to challenge longstanding decisions and policies of FDA, which could undermine FDA's authority, lead to uncertainties in the industry, and disrupt FDA's normal operations, which could delay FDA's review of our marketing applications.

Success in preclinical studies or earlier clinical trials may not be indicative of results in future clinical trials.

Success in preclinical testing and early clinical trials does not ensure that later clinical trials will generate the same results or otherwise provide adequate data to demonstrate the efficacy and safety of a product candidate. Preclinical tests and Phase 1 and Phase 2 clinical trials are primarily designed to test safety, to study pharmacokinetics and pharmacodynamics and to understand the side effects of product candidates at various doses and schedules. Success in preclinical or animal studies and early clinical trials does not ensure that later larger-scale efficacy and safety trials will be successful, nor does it predict final results. For example, we may be unable to identify suitable animal disease models for our product candidates, which could delay or frustrate our ability to proceed into clinical trials or obtain marketing approval. In addition, the preclinical studies conducted by Stelios (an entity that we acquired in 2021) and UCSD for our product candidates LX2021 and LX2022 employed an AAV9-based formulation and studies using this vector may not be predictive of future testing we intend to conduct using an AAVrh10-based formulation or other potential capsid serotypes.

Our product candidates may fail to show the desired safety and efficacy in clinical development despite positive results in preclinical studies or having successfully advanced through initial clinical trials. Furthermore, our currently ongoing and most future clinical trials involve or will involve a small patient population. Because of the small sample sizes studied in our trials thus far, the results of these trials may not be indicative of results of future clinical trials.

Additionally, some of our ongoing and planned clinical trials utilize, or may utilize, an "open-label" trial design. An "open-label" clinical trial is one where both the patient and investigator know whether the patient is receiving the investigational product candidate or either an existing approved drug or placebo. Most typically, open-label clinical trials test only the investigational product candidate and sometimes may do so at different dose levels. Open-label clinical trials are subject to various limitations that may exaggerate any therapeutic effect as patients in open-label clinical trials are aware when they are receiving treatment. Open-label clinical trials may be subject to a "patient bias" where patients perceive their symptoms to have improved merely due to their awareness of receiving an experimental treatment. In addition, open-label clinical trials may be subject to an "investigator bias" where those assessing and reviewing the physiological outcomes of the clinical trials are aware of which patients have received treatment and may interpret the information of the treated group more favorably given this knowledge. The results from an open-label trial may not be predictive of future clinical trial results when studied in a controlled environment with a placebo or active control.

Many companies in the pharmaceutical and biotechnology industries have suffered significant setbacks in late-stage clinical trials even after achieving promising results in preclinical testing and earlier-stage clinical trials. Data obtained from preclinical and clinical activities are subject to varying interpretations, which may delay, limit or prevent regulatory approval. In addition, we may experience regulatory delays or rejections as a result of many factors, including changes in regulatory policy during the period of our product candidate development. Any such delays could negatively impact our business, financial condition, results of operations and prospects.

Interim “top-line” and preliminary results from our clinical trials that we announce, publish or present from time to time may change as more patient data become available and are subject to audit and verification procedures that could result in material changes in the final data.

From time to time, we may publish or present interim top-line or preliminary results from our clinical trials. Interim results from clinical trials that we may complete are subject to the risk that one or more of the clinical outcomes may materially change as patient enrollment continues and more patient data become available. Preliminary or top-line results also remain subject to audit and verification procedures that may result in the final data being materially different from the preliminary data we previously published. As a result, interim and preliminary data should be viewed with caution until the final data are available. Differences between preliminary or interim data and final data could significantly harm our business prospects and may cause the trading price of our Common Stock to fluctuate significantly.

Our preclinical studies and clinical trials may fail to demonstrate the safety and efficacy of our product candidates, or serious adverse or unacceptable side effects may be identified during the development of our product candidates, which could prevent or delay regulatory approval and commercialization, increase our costs or necessitate the abandonment or limitation of the development of some of our product candidates. We may also identify safety and efficacy concerns after the approval of a product candidate which can result in negative consequences to our business and results of operations.

Before obtaining regulatory approvals for the commercial sale of our product candidates, we must demonstrate through lengthy, complex and expensive preclinical testing and clinical trials that our product candidates are safe, effective and of sufficient purity for use in each target disease, and failures can occur at any stage of testing. Preclinical studies and clinical trials often fail to demonstrate safety or efficacy of the product candidate studied for the target disease. While we have developed our AAVrh10-mediated gene therapy product candidates to leverage the low seropositivity of AAVrh10, any gene therapy product based on viral vectors carries the risks of immunogenicity, elevated liver enzymes and insertional oncogenesis, which is the process whereby the insertion of a functional gene near a gene that is important in cell growth or division results in uncontrolled cell division, which could potentially enhance the risk of malignant transformation. In one of our preclinical studies of LX2006, we observed four cases of hepatocellular carcinoma, or HCC, in wild-type mice at 10 months post-treatment. Although data reported by the FDA Cellular, Tissue, and Gene Therapies Advisory Committee in September 2021 suggests that HCC observed in mice after AAV treatment is unlikely to translate to risks for humans, any future instances of HCC in our clinical trials could result in delays or the abandonment of our trials. Health authorities also ask that sponsors closely monitor the risk of elevated liver enzymes and abnormal liver ultrasound on a routine basis in patients participating in gene therapy clinical trials.

Possible adverse side effects that could occur with treatment with gene therapy products include an immunologic reaction early after administration, which, while not necessarily adverse to the patient’s health, could substantially limit the effectiveness of the treatment. For example, in previous third-party clinical trials involving AAV capsids for gene therapy, some subjects experienced the development of a T-cell antibody response, whereby after the vector is within the target cell types, the cellular immune response system triggers the removal of transduced cell types by activated T-cells. If any of our product candidates demonstrate a similar effect, we may decide or be required to perform additional preclinical studies or to halt or delay further clinical development of our product candidates.

In addition to side effects caused by the product candidate, the administration process or related procedures also can cause adverse side effects. Our APOE-associated Alzheimer’s disease product candidates are designed to be delivered via intracisternal administration. While the intracisternal method of administration has been available for some years, its use for gene therapies is new and no gene therapy is currently approved for this method of administration. Intracisternal administration may have greater risk and/or be perceived as having greater risk than more common methods of administration, such as intravenous injection. Other gene therapy product candidates in clinical development utilizing intracisternal delivery could also generate data that could adversely affect the clinical, regulatory or commercial perception of our product candidates.

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If adverse events occur, either as a result of the product candidate or administration process, our clinical trials could be suspended or terminated. If we cannot demonstrate that any adverse events were not caused by the drug or administration process or related procedures, the FDA, EMA or foreign regulatory authorities could order us to cease further development of, or deny approval of, our product candidates for any or all targeted diseases. Even if we are able to demonstrate that all future serious adverse events are not product-related, such occurrences could affect patient recruitment or the ability of enrolled patients to complete the trial. Moreover, if we elect, or are required, to not initiate, delay, suspend or terminate any future clinical trial of any of our product candidates, the commercial prospects of such product candidates may be harmed and our ability to generate product revenues from any of these product candidates may be delayed or eliminated. Any of these occurrences may harm our ability to develop other product candidates, and may harm our business, financial condition and prospects significantly. Furthermore, negative results in our development of LX2006, LX1001, or LX2020 could be interpreted as a failure to achieve proof of concept for our technology and result in the abandonment of other development programs.

In addition, gene therapy is still a relatively new approach to disease treatment and additional adverse side effects could develop. There also is the potential risk of delayed adverse events following exposure to gene therapy products due to persistent biologic activity of the genetic material or other components of products used to carry the genetic material. If our product candidates are associated with side effects in clinical trials or have characteristics that are unexpected, we may need to abandon their development or limit development to more narrow uses in which the side effects or other characteristics are less prevalent, less severe or more acceptable from a risk-benefit perspective. The FDA or an IRB may also require that we suspend, discontinue, or limit our clinical trials based on safety information, or that we conduct additional animal or human studies regarding the safety and efficacy of our product candidates which we have not planned or anticipated. Such findings could further result in regulatory authorities failing to provide marketing authorization for our product candidates or limiting the scope of the approved indication, if approved. Many product candidates that initially showed promise in early-stage testing have later been found to cause side effects that prevented further development of the product candidate.

Additionally, if one or more of our product candidates receives marketing approval, and we or others identify undesirable side effects caused by such products or the administration procedure, a number of potentially significant negative consequences could result, including:

- regulatory authorities may withdraw approvals of such product;
- regulatory authorities may require additional warnings on the labels;
- we may be required to create a medication guide outlining the risks of such side effects for distribution to patients or other requirements subject to a REMS;
- we could be sued and held liable for harm caused to patients;
- we may not be able to obtain or maintain third-party payor coverage and adequate reimbursement; and
- our reputation and physician or patient acceptance of our products may suffer.

There can be no assurance that we will resolve any issues related to any product-related adverse events to the satisfaction of the FDA or foreign regulatory agency in a timely manner or at all. Moreover, any of these events could prevent us from achieving or maintaining market acceptance of the particular product candidate, if approved, and could significantly harm our business, results of operations and prospects.

Some of the diseases we initially seek to treat have low prevalence and it may be difficult to identify and enroll patients with these diseases. If we experience delays or difficulties in the enrollment and/or maintenance of patients in clinical trials, our receipt of necessary regulatory approvals could be delayed or prevented.

Successful and timely completion of clinical trials will require that we enroll a sufficient number of patients. Patient enrollment, a significant factor in the timing of clinical trials, is affected by many factors, including the

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size and nature of the patient population and competition for patients with other trials. The rare genetic diseases which some of our product candidates are designed to target have low incidence and prevalence and may be difficult to diagnose. In particular, because we are focused on patients with specific genetic mutations, our ability to enroll eligible patients may be limited or enrollment may be slower than we anticipate. For example, we estimate that approximately 6,600 people in the United States have FA and that approximately 80% of these patients will develop the cardiac manifestation of FA, or FA cardiomyopathy, and accordingly it may be difficult for us to identify and timely recruit a sufficient number of eligible patients to conduct our clinical trials. While the patient population for LX2020, our program targeting PKP2-ACM, is significantly larger than FA, we may face challenges in identifying and recruiting eligible patients to conduct our clinical trial given competing clinical trials. Even for more prevalent conditions such as Alzheimer's disease, it may be difficult to recruit patients to clinical trials due to the number of approved products, difficulty identifying patients with the specific genotype we are studying, and the number of clinical trials being conducted in this indication.

Our trials may be subject to delays as a result of patient enrollment taking longer than anticipated or patient withdrawal. We may not be able to initiate or continue clinical trials for our product candidates if we are unable to locate and enroll a sufficient number of eligible patients to participate in these trials as required by the FDA, EMA or other foreign regulatory authorities. We cannot predict how successful we will be at enrolling subjects in future clinical trials. Subject enrollment is affected by other factors including:

- the severity of the disease under investigation;
- the eligibility criteria for the trial in question;
- the size of the patient population and process for identifying patients;
- the perceived risks and benefits of the product candidate under study;
- clinicians' and patients' perceptions as to the potential advantages of the product candidate being studied in relation to other available therapies, including any new drugs that may be approved for the diseases we are investigating;
- the availability of competing commercially available therapies and other competing therapeutic product candidates' clinical trials;
- the efforts to facilitate timely enrollment in clinical trials;
- the risk that patients enrolled in clinical trials will drop out of the clinical trials before completion of their treatment;
- the patient referral practices of physicians;
- the ability to monitor patients adequately during and after treatment; and
- the proximity and availability of clinical trial sites for prospective patients.

Our inability to enroll a sufficient number of patients for clinical trials would result in significant delays and could require us to abandon one or more clinical trials altogether. Enrollment delays in these clinical trials may result in increased development costs for our product candidates, which would cause the value of our company to decline and limit our ability to obtain additional financing. Furthermore, we rely on CROs and clinical trial sites to help ensure the proper and timely conduct of our clinical trials and we may have limited influence over their performance. For additional information, see the risk factor in this section under the heading "*We intend to continue to rely on third parties to conduct a significant portion of our existing clinical trials and potential future clinical trials for product candidates, and those third parties may not perform satisfactorily, including failing to meet deadlines for the completion of such trials.*"

Furthermore, even if we are able to enroll a sufficient number of patients for our clinical trials, we may have difficulty maintaining enrollment of such patients in our clinical trials.

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We may seek Orphan Drug designation or Rare Pediatric Disease designation for some of our product candidates and we may be unsuccessful, or may be unable to maintain the benefits associated with Orphan Drug designation, including the potential for market exclusivity, for product candidates for which we obtain Orphan Drug designation.

Regulatory authorities in some jurisdictions, including the United States, may designate drugs or biologics intended to treat relatively small patient populations as orphan drug products. Under the Orphan Drug Act, the FDA may designate a drug or biologic as an orphan drug if it is intended to treat a rare disease or condition, which is generally defined as a patient population of fewer than 200,000 individuals in the United States, or a patient population of 200,000 or more in the United States where there is no reasonable expectation that the cost of developing the drug will be recovered from sales in the United States.

In the United States, Orphan Drug designation entitles a party to potential financial incentives such as tax advantages and user fee waivers. Opportunities for grant funding toward clinical trial costs may also be available for clinical trials of drugs or biologics for rare diseases, regardless of whether the drugs or biologics are designated for the orphan use. In addition, if a drug or biologic with an Orphan Drug designation subsequently receives the first marketing approval for the disease for which it has such designation, the product is entitled to a seven-year period of marketing exclusivity, which precludes the FDA from approving another marketing application for the same drug and disease for that time period, except in limited circumstances. If our competitors are able to obtain orphan drug exclusivity prior to us, for products that constitute the “same drug” and treat the same diseases as our product candidates, we may not be able to have competing products approved by the applicable regulatory authority for a significant period of time.

Similarly, in the European Union, the European Commission, upon the recommendation of the EMA’s Committee for Orphan Medicinal Products, grants Orphan Drug designation to promote the development of drugs that are intended for the diagnosis, prevention or treatment of life-threatening or chronically debilitating conditions and either the prevalence of the condition is not more than 5 in 10,000 persons in the European Union, or, without incentives, it is unlikely that sales of the drug in the European Union would be sufficient to justify the necessary investment in developing the drug. In each case, there must be no satisfactory method of diagnosis, prevention or treatment of the condition that has been authorized, or, if such a method exists, the product in question must be of significant benefit to those affected by such condition. In the European Union, Orphan Drug designation entitles a party to financial incentives such as reduction of fees or fee waivers.

We have obtained from the FDA Orphan Drug designation for LX2006 for treatment of FA and for LX2020 for the treatment of PKP2-ACM. We may seek orphan designation for some or all of our product candidates in orphan indications in which there is a medically plausible basis for the use of these product candidates. However, we may be unsuccessful in obtaining Orphan Drug designation and may be unable to maintain the benefits associated with such designations. Even if we obtain orphan drug exclusivity for any of our product candidates, that exclusivity may not effectively protect those product candidates from competition because different drugs can be approved for the same condition, and orphan drug exclusivity does not prevent the FDA from approving the same or a different drug in another indication. Even after an orphan drug is granted orphan exclusivity and approved, the FDA can subsequently approve a later application for the same drug for the same condition before the expiration of the seven-year exclusivity period if the FDA concludes that the later drug is clinically superior in that it is shown to be safer in a substantial portion of the target populations, more effective or makes a major contribution to patient care. On August 3, 2017, Congress passed the FDA Reauthorization Act of 2017, or FDARA. FDARA, among other things, codified the FDA’s preexisting regulatory interpretation, to require that a drug sponsor demonstrate the clinical superiority of an orphan drug that is otherwise the same as a previously approved drug for the same rare disease in order to receive orphan drug exclusivity. The statute supplants prior precedent holding that the Orphan Drug Act unambiguously requires that the FDA recognize the orphan exclusivity period regardless of a showing of clinical superiority. Moreover, in the Consolidated Appropriations Act of 2021, Congress clarified that the interpretation of orphan drug exclusivity codified in FDARA would apply in cases where the FDA issued an orphan designation before the enactment of FDARA but where product

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approval came after the enactment of FDARA. In addition, a designated Orphan Drug may not receive orphan drug exclusivity if it is approved for a use that is broader than the disease for which it received orphan designation. On January 24, 2023, the FDA announced its intention to apply its existing regulations and long-standing approach to grant orphan drug exclusivity based on the indications for which the drug is approved rather than granting the exclusivity for the entire rare disease or condition that was the subject of the orphan drug designation, in response to the U.S. Court of Appeals for the Eleventh Circuit's September 30, 2021, decision in *Catalyst Pharms., Inc. v. Becerra*. The FDA may further reevaluate its regulations and policies under the Orphan Drug Act. We do not know if, when, or how the FDA, Congress, or future judicial challenges may change the orphan drug regulations and policies in the future, and it is uncertain how any changes might affect our business. Depending on what changes the FDA may make to its orphan drug regulations and policies, our business could be adversely impacted. Moreover, orphan drug-exclusive marketing rights in the United States may be lost if the FDA later determines that the request for designation was materially defective or if we are unable to manufacture sufficient quantities of the product to meet the needs of patients with the rare disease or condition. Orphan Drug designation neither shortens the development time or regulatory review time of a drug nor gives the drug any advantage in the regulatory review or approval process.

Fast Track, Breakthrough Therapy, or Regenerative Medicine Advanced Therapy designation that we may receive from FDA may not actually lead to a faster development or regulatory review or approval process, and does not assure FDA approval of our product candidates.

We may seek Fast Track, Breakthrough Therapy or RMAT designation from the FDA for some or all of our product candidates, but we may be unable to obtain such designations or to maintain the benefits associated with such designations. The FDA's Fast Track, Breakthrough Therapy, and RMAT designation programs are intended to expedite the development of certain qualifying product candidates intended for the treatment of serious diseases and conditions. If a product candidate is intended for the treatment of a serious or life-threatening condition and preclinical or clinical data demonstrate the product's potential to address an unmet medical need for this condition, the sponsor may apply for FDA Fast Track designation.

A product candidate may be designated as a breakthrough therapy if it is intended, alone or in combination with one or more other drugs or biologics to treat a serious or life-threatening condition and preliminary clinical evidence indicates that the product candidate may demonstrate substantial improvement over existing therapies on one or more clinically significant endpoints. For product candidates that have been designated as breakthrough therapies, interaction and communication between the FDA and the sponsor of the trial can help to identify the most efficient path for clinical development while minimizing the number of patients placed in ineffective control regimens. Drugs and biologics designated as breakthrough therapies by the FDA may also be eligible for accelerated approval.

A product candidate may receive RMAT designation if it is a regenerative medicine therapy that is intended to treat, modify, reverse or cure a serious or life-threatening disease or condition, and preliminary clinical evidence indicates that the product candidate has the potential to address an unmet medical need for such condition. RMAT designation allows companies developing regenerative medicine therapies to work more closely and frequently with the FDA, and RMAT-designated product candidates may be eligible for priority review and accelerated approval. FDA has confirmed that gene therapies, including genetically modified cells, that lead to a sustained effect on cells or tissues may meet the definition of a regenerative medicine therapy. For product candidates that have received an RMAT designation, interaction and communication between the FDA and the sponsor of the trial can help to identify the most efficient path for clinical development while minimizing the number of patients placed in ineffective control regimens.

We have received Fast Track designation for LX1001 for the treatment of patients with early Alzheimer's disease who are *APOE4* homozygous, to slow disease progression and have received Fast Track designation for LX2020 for the treatment of PKP2-ACM. While we may seek Fast Track, Breakthrough Therapy and/or RMAT designation for some or all of our product candidates, there is no guarantee that we will be successful in

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obtaining any such designation. Even if we do obtain such designation, we may not experience a faster development process, review or approval compared to conventional FDA procedures. A Fast Track, Breakthrough Therapy, or RMAT designation does not ensure that the product candidate will receive marketing approval or that approval will be granted within any particular time frame. In addition, the FDA may withdraw Fast Track, Breakthrough Therapy, or RMAT designation if it believes that the designation is no longer supported by data from our clinical development program. Fast Track, Breakthrough Therapy and/or RMAT designation alone does not guarantee qualification for the FDA's priority review procedures.

We have received Rare Pediatric Disease designation from the FDA for LX2006 for the treatment of FA and we may seek such designation for future product candidates. However, a marketing application for these product candidates, if approved, may not meet the eligibility criteria for a rare pediatric disease priority review voucher.

We have received Rare Pediatric Disease designation from the FDA for LX2006 for the treatment of FA and LX1004 for the treatment of CLN2 disease and we may seek Rare Pediatric Disease designation for future product candidates. The FDA defines "rare pediatric disease" as a (i) serious or life-threatening disease in which the serious or life-threatening manifestations primarily affect individuals aged from birth to 18 years, including age groups often called neonates, infants, children, and adolescents; and (ii) a rare disease or condition within the meaning of the Orphan Drug Act. Designation of a product candidate as a product for a rare pediatric disease does not guarantee that a marketing application for such product candidate will meet the eligibility criteria for a rare pediatric disease priority review voucher at the time the application is approved. Under the FDCA, we will need to request a rare pediatric disease priority review voucher in our original marketing application for our product candidates for which we have received Rare Pediatric Disease designation. The FDA may determine that a marketing application for any such product candidates, if approved, does not meet the eligibility criteria for a priority review voucher, including for the following reasons:

- the rare pediatric disease that received such designation no longer meets the definition of a "rare pediatric disease";
- the marketing application contains an active ingredient (including any ester or salt of the active ingredient) that has been previously approved in a marketing application
- the marketing application is not deemed eligible for priority review
- the marketing application does not rely on clinical data derived from studies examining a pediatric population and dosages of the product intended for that population (that is, if the marketing application does not contain sufficient clinical data to allow for adequate labeling for use by the full range of affected pediatric patients); or
- the marketing application is approved for a different adult indication than the rare pediatric disease for which our product candidates are designated.

Under the current statutory sunset provisions, after September 30, 2024, the FDA may only award a priority review voucher, or PRV, for an approved rare pediatric disease product application if the sponsor has Rare Pediatric Disease designation for the drug or biologic that is the subject of such application, and that designation was granted by September 30, 2024. After September 30, 2026, the FDA may not award any rare pediatric disease priority review vouchers. However, it is possible the authority for FDA to award rare pediatric disease priority review vouchers will be further extended by Congress. As such, if we do not obtain approval of a marketing application for LX2006 in patients with FA on or before September 30, 2026, and if the priority review voucher program is not extended by Congressional action, we may not receive a priority review voucher.

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Where appropriate, we may seek approval from the FDA, EMA or comparable foreign regulatory authorities through the use of accelerated approval pathways. If we are unable to obtain such approval, we may be required to conduct additional preclinical studies or clinical trials beyond those that we contemplate, which could increase the expense of obtaining, and delay the receipt of, necessary marketing approvals. Even if we receive accelerated approval from the FDA, EMA or comparable regulatory authorities, if our confirmatory trials do not verify clinical benefit, or if we do not comply with rigorous post-marketing requirements, the FDA, EMA or such other regulatory authorities may seek to withdraw accelerated approval.

Where possible, we may pursue accelerated development strategies in areas of high medical need. We may seek an accelerated approval pathway for one or more of our therapeutic product candidates from the FDA, EMA or comparable foreign regulatory authorities. Under the accelerated approval provisions in the FDCA and the FDA's implementing regulations, the FDA may grant accelerated approval to a therapeutic candidate that is designed to treat a serious or life-threatening condition, generally provides a meaningful therapeutic benefit over available therapies, and demonstrates an effect on a surrogate endpoint that is reasonably likely to predict clinical benefit or on a clinical endpoint that can be measured earlier than irreversible morbidity or mortality, or IMM, that is reasonably likely to predict an effect on IMM or other clinical benefit. The FDA considers a clinical benefit to be a positive therapeutic effect that is clinically meaningful in the context of a given disease, such as IMM. For the purposes of accelerated approval, a surrogate endpoint is a marker, such as a laboratory measurement, radiographic image, physical sign, or other measure that is thought to predict clinical benefit, but is not itself a measure of clinical benefit. An intermediate clinical endpoint is a clinical endpoint that can be measured earlier than an effect on IMM that is reasonably likely to predict an effect on IMM or other clinical benefit. The accelerated approval pathway may be used in cases in which the advantage of a new product over available therapy may not be a direct therapeutic advantage, but is a clinically important improvement from a patient and public health perspective. If granted, accelerated approval is usually contingent on the sponsor's agreement to conduct, in a diligent manner, additional post-approval confirmatory studies to verify and describe the product's clinical benefit. If such post-approval studies fail to confirm the product's clinical benefit, the FDA may withdraw its approval of the product. In addition, the FDA currently requires, unless otherwise informed by the agency, pre-approval of promotional materials for products receiving accelerated approval, which could adversely impact the timing of the commercial launch of the product.

Prior to seeking accelerated approval, we would seek feedback from the FDA, EMA or comparable foreign regulatory authorities and would otherwise evaluate our ability to seek and receive such accelerated approval. There can be no assurance that after our evaluation of the feedback and other factors we will decide to pursue or submit a BLA for accelerated approval or any other form of expedited development, review or approval. Similarly, there can be no assurance that after subsequent feedback from the FDA, EMA or comparable foreign regulatory authorities, we will continue to pursue or apply for accelerated approval or any other form of expedited development, review or approval, even if we initially decide to do so. Furthermore, if we decide to submit an application for accelerated approval, there can be no assurance that such application will be accepted or that any approval will be granted on a timely basis, or at all. The FDA, EMA or other comparable foreign regulatory authorities could also require us to conduct further studies prior to considering our application or granting approval of any type, including, for example, if other products are approved via the accelerated pathway and subsequently converted by FDA to full approval. A failure to obtain accelerated approval or any other form of expedited development, review or approval for our therapeutic candidate would result in a longer time period to commercialization of such therapeutic candidate, could increase the cost of development of such therapeutic candidate and could harm our competitive position in the marketplace.

Priority review designation by the FDA may not lead to a faster regulatory review or approval process and, in any event, does not assure FDA approval of our product candidates.

If the FDA determines that a product candidate is intended to treat a serious disease or condition and, if approved, would provide a significant improvement in the safety or effectiveness of the treatment, prevention, or diagnosis of such disease or condition, the FDA may designate the product candidate for priority review. A

priority review designation means that the goal for the FDA to review a marketing application is six months from filing of the application, rather than the standard review period of ten months. We may request priority review for certain of our product candidates. The FDA has broad discretion with respect to whether or not to grant priority review status to a product candidate, so even if we believe a particular product candidate is eligible for such designation or status, the FDA may disagree and decide not to grant it. Moreover, a priority review designation does not necessarily mean a faster regulatory review process or necessarily confer any advantage with respect to approval compared to conventional FDA procedures. Receiving priority review from the FDA does not guarantee approval within the six-month review cycle or thereafter.

We may expend our limited resources to pursue a particular product candidate and fail to capitalize on product candidates that may be more profitable or for which there is a greater likelihood of success.

Because we have limited financial and management resources, we must focus on development programs and product candidates that we identify for specific diseases. As such, currently we are primarily focused on the development of our current pipeline of product candidates. As a result, we may forego or delay pursuit of opportunities with other product candidates. For example, we are evaluating strategic alternatives to find the appropriate partner to advance our LX1004 program. Our resource allocation decisions may cause us to fail to capitalize on viable commercial products or profitable market opportunities. Our spending on current and future development programs and product candidates for specific diseases may not yield any commercially viable products. If we do not accurately evaluate the commercial potential or target market for a particular product candidate, we may relinquish valuable rights to that product candidate through collaboration, licensing or other royalty arrangements in cases in which it would have been more advantageous for us to retain sole development and commercialization rights to such product candidate.

We may not be successful in our efforts to build a pipeline of additional product candidates.

Our business model is centered on developing product candidates targeting patient populations that place significant burden on society and are most amenable to our genetic medicine approach. We are targeting diseases that have seen limited penetration of precision medicine and where we believe there is significant opportunity for gene therapy to play a role as a key therapeutic option. We aim to select, develop and advance product candidates that we believe will have a high probability of technical and regulatory success through development into commercialization. We may not be able to continue to identify and develop new product candidates. Even if we are successful in continuing to build our pipeline, the potential product candidates that we identify may not be suitable for clinical development. For example, they may be shown to have side effects or other characteristics that indicate that they are unlikely to be drugs that will receive marketing approval and achieve market acceptance. If we do not successfully develop and commercialize product candidates based upon our approach, we will not be able to obtain product revenue in future periods, which likely would result in significant harm to our financial position and adversely affect our stock price.

If we are unable to successfully validate, develop and obtain regulatory approval for companion diagnostic tests for our product candidates that require or would commercially benefit from such tests, or experience significant delays in doing so, we may not realize the full commercial potential of these product candidates.

In connection with the clinical development of our product candidates for certain indications, we intend to work with collaborators to develop or obtain access to *in vitro* companion diagnostic tests to identify patient subsets within a disease category who may derive selective and meaningful benefit from our product candidates. To be successful, we or our collaborators will need to address a number of scientific, technical, regulatory and logistical challenges. The FDA and comparable foreign regulatory authorities regulate *in vitro* companion diagnostics as medical devices and, under that regulatory framework, will likely require the conduct of clinical trials to demonstrate the safety and effectiveness of any diagnostics we may develop, which we expect will require separate regulatory clearance or approval prior to commercialization. The FDA generally will require approval or clearance of the diagnostic at the same time that the FDA approves the therapeutic product if the

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FDA determines that safe and effective use of a therapeutic product depends on an *in vitro* companion diagnostic. The clearance or approval of a companion diagnostic as part of the product label will also limit the use of the product candidate to patients who have met the screening criteria tested for by the companion diagnostic.

We intend to rely on third parties for the design, development and manufacture of companion diagnostic tests for our therapeutic product candidates that may require such tests. If we enter into such collaborative agreements, we will be dependent on the sustained cooperation and effort of our future collaborators in developing and obtaining approval for these companion diagnostics. It may be necessary to resolve issues such as selectivity/specificity, analytical validation, reproducibility, or clinical validation of companion diagnostics during the development and regulatory approval processes. Moreover, even if data from preclinical studies and early clinical trials appear to support development of a companion diagnostic for a product candidate, data generated in later clinical trials may fail to support the analytical and clinical validation of the companion diagnostic. We and our future collaborators may encounter difficulties in developing, obtaining regulatory approval for, manufacturing and commercializing companion diagnostics similar to those we face with respect to our therapeutic candidates themselves, including issues with achieving regulatory clearance or approval, production of sufficient quantities at commercial scale and with appropriate quality standards, and in gaining market acceptance. If we are unable to successfully develop companion diagnostics for these product candidates, or experience delays in doing so, the development of these product candidates may be adversely affected, these product candidates may not obtain marketing approval, and we may not realize the full commercial potential of any of these product candidates that obtain marketing approval. As a result, our business, results of operations and financial condition could be materially harmed. In addition, a diagnostic company with whom we contract may decide to discontinue selling or manufacturing the companion diagnostic test that we anticipate using in connection with development and commercialization of our product candidates or our relationship with such diagnostic company may otherwise terminate. We may not be able to enter into arrangements with another diagnostic company to obtain supplies of an alternative diagnostic test for use in connection with the development and commercialization of our product candidates or do so on commercially reasonable terms, which could adversely affect and/or delay the development or commercialization of our product candidates.

Risks related to the manufacturing of our product candidates

We and our contract manufacturers are subject to significant regulation with respect to manufacturing our products. The third-party manufacturing facilities on which we rely, and any manufacturing facility that we may have in the future, may have limited capacity or fail to meet the applicable stringent regulatory requirements.

We currently have relationships with a limited number of suppliers for the manufacturing of all components of our product candidates. However, if we experience slowdowns or problems with our manufacturing partners and are unable to establish or scale our internal manufacturing capabilities, we will need to continue to contract with manufacturers that can produce the preclinical, clinical and commercial supply of our products. Each supplier may require licenses to manufacture such components if such processes are not owned by the supplier or in the public domain and we may be unable to license such intellectual property rights on reasonable commercial terms or to transfer or sublicense the intellectual property rights we may have with respect to such activities.

All entities involved in the preparation of therapeutics for clinical trials or commercial sale, including our existing contract manufacturers for components of our product candidates, are subject to extensive regulation. Components of a finished therapeutic product approved for commercial sale or used in late-stage clinical trials in the United States and European Union must be manufactured in accordance with cGMP. These regulations govern manufacturing processes and procedures (including recordkeeping) and the implementation and operation of quality systems to control and assure the quality of investigational products and products approved for sale. Poor control of production processes can lead to the introduction of adventitious agents or other contaminants, or to inadvertent changes in the properties or stability of our product candidates that may not be detectable in final product testing. We or our contract manufacturers must supply all necessary documentation in support of a BLA

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or an MAA on a timely basis. Our potential manufacturing facilities and quality systems and the facilities and quality systems of some or all of our third-party contractors must pass a pre-approval inspection for compliance with the applicable regulations as a condition of regulatory approval of our product candidates or any of our other potential products. In addition, the regulatory authorities may, at any time, audit or inspect a manufacturing facility involved with the preparation of our product candidates or our other potential products or the associated quality systems for compliance with the regulations applicable to the activities being conducted, and they could put a hold on one or more of our clinical trials if the facilities of our CMOs do not pass such audit or inspections. If these facilities do not pass a pre-approval plant inspection, FDA approval of the products will not be granted.

The regulatory authorities also may, at any time following approval of a product for sale, inspect or audit our manufacturing facilities or those of our third-party contractors. If any such inspection or audit identifies a failure to comply with applicable regulations or if a violation of our product specifications or applicable regulations occurs independent of such an inspection or audit, we or the relevant regulatory authority may require remedial measures that may be costly and/or time-consuming for us or a third party to implement and that may include the temporary or permanent suspension of a clinical trial or commercial sales or the temporary or permanent closure of a facility. Any such remedial measures imposed upon us or third parties with whom we contract could harm our business.

If we or any of our third-party manufacturers fail to maintain regulatory compliance, the FDA can impose regulatory sanctions including, among other things, refusal to approve a pending application for a new drug product or biologic product, or revocation of a pre-existing approval. As a result, our business, financial condition and results of operations may be harmed. Additionally, if supply from one approved manufacturer is interrupted, there could be a significant disruption in commercial supply. An alternative manufacturer would need to be qualified through a BLA and/or an MAA supplement which could result in further delay. The regulatory agencies may also require additional studies if a new manufacturer is relied upon for commercial production. Switching manufacturers may involve substantial costs and is likely to result in a delay in our desired clinical and commercial timelines.

These factors could cause the delay of clinical trials, regulatory submissions, required approvals or commercialization of our product candidates, cause us to incur higher costs and prevent us from commercializing our products successfully, if approved. Furthermore, if our suppliers fail to meet contractual requirements, and we are unable to secure one or more replacement suppliers capable of production at a substantially equivalent cost, our clinical trials may be delayed, or we could lose potential revenue.

Gene therapies are novel, complex and difficult to manufacture. We could experience manufacturing problems that result in delays in the development or commercialization of our product candidates or otherwise harm our business.

The manufacture of gene therapy products is technically complex and necessitates substantial expertise and capital investment. Production difficulties caused by unforeseen events may delay the availability of material for our clinical studies.

We rely on third-party manufacturers to manufacture our product candidates for preclinical studies and clinical trials. The manufacturers of pharmaceutical products must comply with strictly enforced cGMP requirements, state and federal regulations, as well as foreign requirements when applicable. Any failure of us or our CMOs to adhere to or document compliance to such regulatory requirements could lead to a delay or interruption in the availability of our product candidate materials for clinical trials or enforcement action from the FDA, EMA or foreign regulatory authorities. If we or our manufacturers fail to comply with the requirements of the FDA, EMA or other regulatory authority, sanctions could be imposed on us, including clinical holds, fines, injunctions, civil penalties, delays, suspension or withdrawal of approvals, license revocation, seizures or recalls of product candidates or products, operating restrictions and criminal prosecutions, any of which could significantly and adversely affect supplies of our product candidates.

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There can be no assurances that our third-party manufacturers will be able to meet our timetable and requirements. If any third party with whom we contract fails to perform its obligations, we may be forced to either manufacture the materials ourselves, for which we may not have the capabilities or resources, or enter into an agreement with a different third-party manufacturer, which we may not be able to do on reasonable terms, if at all. In either scenario, our clinical trials and future commercial supply could be delayed significantly as we establish alternative supply sources. In some cases, the technical skills required to manufacture our product candidates may be unique or proprietary to the original third-party manufacturer and we may have difficulty, or there may be contractual restrictions prohibiting us from, transferring such skills to a back-up or alternate supplier, or we may be unable to transfer such skills at all. In addition, if we are required to change manufacturers for any reason, we will be required to verify that the new manufacturer maintains facilities and procedures that comply with quality standards and with all applicable regulations. We will also need to verify, such as through a manufacturing comparability study, that any new manufacturing process will produce our product candidates according to the specifications previously submitted to the FDA or another regulatory authority. The delays associated with the verification of a new manufacturer could negatively affect our ability to develop product candidates or commercialize our products, if approved, in a timely manner or within budget.

If we are unable to arrange for alternative third-party manufacturing sources on commercially reasonable terms or in a timely manner, we may be delayed in the development of our product candidates. Our dependence upon others for the manufacture of our product candidates may also adversely affect our future profit margins and our ability to commercialize any product candidates that receive regulatory approval on a timely and competitive basis.

Our product candidates require processing steps that are more complex than those required for most chemical pharmaceuticals. Moreover, unlike chemical pharmaceuticals, the physical and chemical properties of a biologic such as our modified virus generally cannot be fully characterized. As a result, assays of the finished product may not be sufficient to ensure that the product will perform in the intended manner. Although we believe that the manufacture of our product candidates may be simplified due to their shared raw materials and other similarities, we cannot be certain that this will be the case and we may be required to develop manufacturing methods that ultimately differ significantly between product candidates, which would require that we invest substantial time and capital to develop suitable manufacturing methods. Our program materials are manufactured using technically complex processes requiring specialized equipment and facilities, highly specific raw materials, cell types and reagents, and other production constraints. Our production process also requires a number of highly specific raw materials, cell types and reagents with limited suppliers. Even though we aim to have backup supplies of raw materials, cell types and reagents whenever possible, we cannot be certain they will be sufficient if our primary sources are unavailable. A shortage of a critical raw material, cell line, or reagent, or a technical issue during manufacturing may lead to delays in clinical development or commercialization plans. We are particularly susceptible to any shortages, delays or our inability to obtain suitable raw materials for our lead product candidates. Any changes in the manufacturing of components of the raw materials we use could result in unanticipated or unfavorable effects in our manufacturing processes, resulting in delays.

In addition, if any of our product candidates obtain approval, the FDA, EMA and other regulatory authorities may require us to submit samples of any lot of any approved product together with the protocols showing the results of applicable tests at any time. Under some circumstances, the FDA, EMA or other regulatory authorities may require that we not distribute a lot until the agency authorizes its release. Slight deviations in the manufacturing process, including those affecting quality attributes and stability, may result in unacceptable changes in the product that could result in lot failures or product recalls. Lot failures or product recalls could cause us to delay product launches or clinical trials, which could be costly to us and otherwise harm our business, financial condition, results of operations and prospects.

We depend on third-party suppliers for materials used in the manufacture of our product candidates, and the loss of these third-party suppliers or their inability to supply us with adequate materials could harm our business.

We rely on third-party suppliers for the materials and components required for the production of our product candidates. Our dependence on these third-party suppliers and the challenges we may face in obtaining adequate supplies of materials involve several risks, including limited control over pricing, availability, and delivery schedules. There is substantial demand and limited supply for certain of the raw materials used to manufacture gene therapy products. As a small company, our negotiation leverage is limited, and we may get lower priority than our competitors that are larger than we are. We cannot be certain that our suppliers will continue to provide us with the quantities of these raw materials within the timelines that we require or satisfy our anticipated specifications and quality requirements. Any supply interruption in limited or sole-sourced raw materials could materially harm our ability to manufacture our product candidates until a new source of supply, if any, could be identified and qualified. We may be unable to find a sufficient alternative supply channel in a reasonable time or on commercially reasonable terms. Any performance failure on the part of our suppliers could delay the development and potential commercialization of our product candidates, including limiting supplies necessary for clinical trials and regulatory approvals, which would have a material adverse effect on our business.

Any contamination or interruption in our manufacturing process, shortages of raw materials or failure of our suppliers of viruses to deliver necessary components could result in delays in our clinical development or marketing schedules.

Given the nature of gene therapy manufacturing, there is a risk of contamination occurring during the manufacturing process. Any contamination could adversely affect our ability to produce product candidates on schedule and could, therefore, harm our results of operations and cause reputational damage. Some of the raw materials required in our manufacturing process are derived from biologic sources. Such raw materials are difficult to procure and may be subject to contamination or recall. A material shortage, contamination, recall or restriction on the use of biologically derived substances in the manufacture of our product candidates could adversely impact or disrupt the commercial manufacturing or the production of clinical material, which could adversely affect our development timelines and our business, financial condition, results of operations and prospects.

Changes in methods of product candidate manufacturing or formulation may result in additional costs or delay.

As product candidates proceed through preclinical studies to late-stage clinical trials towards potential approval and commercialization, it is common that various aspects of the development program, such as manufacturing methods and formulation, are altered along the way in an effort to optimize processes and product characteristics. Such changes carry the risk that they will not achieve our intended objectives. Any such changes could cause our product candidates to perform differently and affect the results of planned clinical trials or other future clinical trials conducted with the materials manufactured using altered processes. Such changes may also require additional testing, FDA notification or FDA approval. This could delay the initiation and completion of clinical trials, require the conduct of bridging clinical trials or the repetition of one or more clinical trials, increase clinical trial costs, delay approval of our product candidates and jeopardize our ability to commence sales and generate revenue. In addition, we may be required to make significant changes to our upstream and downstream processes across our pipeline, which could delay the development of our future product candidates. Regulatory agencies, and in particular the FDA and EMA, have demonstrated increased caution in their regulation of gene therapies, including increased scrutiny related to chemistry, manufacturing and control, or CMC, issues. This increased regulatory scrutiny around gene therapy CMC may result in us being required to conduct additional preclinical studies or clinical trials with respect to any of our product candidates, which may result in delays and increased costs in the development or commercialization of our product candidates and ultimately could lead to the failure to obtain approval for any gene therapy product.

Risks related to the commercialization of our product candidates

Even if any of our product candidates receive marketing approval, they may fail to achieve the degree of market acceptance by physicians, patients, third-party payors and others in the medical community necessary for commercial success.

If any of our product candidates receive marketing approval, they may nonetheless fail to gain sufficient market acceptance by physicians, patients, third-party payors and others in the medical community. If our product candidates do not achieve an adequate level of acceptance, we may not generate significant revenue and we may not become profitable. The degree of market acceptance of our product candidates, if approved for commercial sale, will depend on a number of factors, including:

- their efficacy, safety and potential advantages compared to alternative treatments;
- our ability to offer our products for sale at competitive prices;
- their convenience and ease of administration compared to alternative treatments;
- product labeling or product insert requirements of the FDA or foreign regulatory authorities, including any limitations or warnings contained in a product's approved labeling, including any boxed warning or REMS;
- the willingness of the target patient population to try new treatments, such as gene therapy as a novel modality for treatment of our target indications and of physicians to prescribe these treatments;
- our ability to hire and retain a sales force in the United States;
- the strength of marketing and distribution support;
- the availability of coverage and adequate reimbursement for our product candidates, once approved, from third-party payors and government authorities;
- the prevalence and severity of any side effects; and
- any restrictions on the use of our products together with other medications.

Negative public opinion of gene therapy and increased regulatory scrutiny of gene therapy and genetic research may adversely impact the development or commercial success of our current and future product candidates.

Our product candidates involve introducing genetic material into a patient's cells via intrathecal and intravenous administration. The clinical and commercial success of our potential products will depend in part on public acceptance of the use of gene therapy and gene regulation for the prevention or treatment of human diseases. Public attitudes may be influenced by claims that gene therapy and gene regulation are unsafe, unethical or immoral, and consequently, our products may not gain the acceptance of the public or the medical community. Adverse public attitudes may adversely impact our ability to enroll clinical trials. Moreover, our success will depend upon physicians prescribing, and their patients being willing to receive, treatments that involve the use of product candidates we may develop in lieu of, or in addition to, existing treatments with which they are already familiar and for which greater clinical data may be available.

More restrictive government regulations or negative public opinion would have a negative effect on our business or financial condition and may delay or impair the development and commercialization of our product candidates or demand for any products once approved. In recent years, sponsors of other clinical trials involving gene therapies have announced imposition of clinical holds by the FDA to evaluate safety issues arising during the trials. Among the risks in any gene therapy product based on viral vectors are the risks of immunogenicity, elevated liver enzymes and insertional oncogenesis. If any of our vectors demonstrate a similar effect, we may decide or be required to halt or delay further clinical development of any product candidates that utilize that vector. Adverse events in our or others' clinical trials, even if not ultimately attributable to our product

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candidates, and the resulting publicity could result in increased governmental regulation, unfavorable public perception, potential regulatory delays in the testing or approval of our product candidates, stricter labeling requirements for those product candidates that are approved and a decrease in demand for any such product candidates. The risk of cancer remains a concern for gene therapy, and we cannot assure that it will not occur in any of our planned or future clinical trials or in any clinical trials conducted by other companies. In addition, there is the potential risk of delayed adverse events following exposure to gene therapy products due to persistent biological activity of the genetic material or other components of products used to carry the genetic material. In addition, for our regulated gene replacement therapy product candidates which require that the expression of a therapeutic transgene be tightly regulated, we may inadvertently cause overexpression, which could lead to numerous issues, including safety and toxicity concerns. Furthermore, one of our regulatory gene replacement therapy candidates, LX1020, requires the insertion of miRNA targets into the viral genome, which is a technology that to our knowledge is not present in any approved gene therapy products. If any such adverse events occur, commercialization of our product candidates or further advancement of our clinical trials could be halted or delayed, which would have a negative impact on our business and operations.

The affected populations for our other product candidates may be smaller than we or third parties currently project, which may affect the addressable markets for our product candidates.

We currently focus our research and product development on several indications that are larger-rare diseases. However, our projections of the number of people who have the diseases we are seeking to treat, as well as the subset of people with these diseases who have the potential to benefit from treatment with our product candidates, are estimates based on our knowledge and understanding of these diseases. These estimates may prove to be incorrect and new studies may further reduce the estimated incidence or prevalence of this disease. The number of patients in the United States, the European Union and elsewhere may turn out to be lower than expected, may not be otherwise amenable to treatment with our product candidate or patients may become increasingly difficult to identify and access, all of which would adversely affect our business, financial condition, results of operations and prospects.

The total addressable market opportunity for our product candidates will ultimately depend upon a number of factors, including the diagnosis and treatment criteria included in the final label, if approved for sale in specified indications, acceptance by the medical community, patient access and product pricing and reimbursement. Incidence and prevalence estimates are frequently based on information and assumptions that are not exact and may not be accurate, and the methodology is forward-looking and potentially speculative. The process we have used in developing an estimated incidence and prevalence range for the indications we are targeting has involved collating limited data from multiple sources. Accordingly, the incidence and prevalence estimates included in this prospectus should be viewed in that context. Further, the data and statistical information used in this prospectus, including estimates derived from them, may differ from information and estimates made by our competitors or from current or future studies conducted by independent sources.

We face significant competition from other biotechnology and pharmaceutical companies, and our operating results will suffer if we fail to compete effectively.

Drug development, particularly in the gene therapy field, is highly competitive and subject to rapid and significant technological advancements. As a significant unmet medical need exists in the cardiovascular disease and Alzheimer's disease areas, there are several large and small pharmaceutical companies focused on delivering therapeutics for the treatment of these diseases, including those that we are initially targeting. It is likely that additional drugs will become available in the future for the treatment of our target diseases.

We are aware that our competitors are developing product candidates for the treatment of diseases that our product candidates will target. With respect to LX2006, we are aware of preclinical gene therapy programs in development at Solid Biosciences Inc. and Lacerta Therapeutics, Inc. and those being developed in collaborations between Voyager Therapeutics, Inc. and Neurocrine Biosciences, Inc. Additionally, we are aware that Prime

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Medicine, Inc. and Tune Therapeutics, Inc. have early-stage gene editing discovery efforts. Among other treatment modalities for FA, we are aware that Larimar Therapeutics, Inc. is developing a clinical stage product candidate, CTI-1601, that Design Therapeutics, Inc. is developing a product candidate, DT-216P2, and that Reata Pharmaceuticals, Inc.'s omaveloxolone (Skyclarys) was approved by the FDA in 2023. In 2023 Biogen Inc. acquired Reata Pharmaceuticals, Inc. for approximately \$7.3 billion and is currently commercializing Skyclarys.

With respect to LX2020, both Rocket and Tenaya Therapeutics Inc. are developing an AAV-based gene therapy candidate designed to deliver a functional *PKP2* gene to patients with PKP2-ACM.

With respect to our portfolio of gene therapy programs for the treatment of homozygous APOE4-associated Alzheimer's disease, we are aware that uniQure, N.V. is pursuing AMT-240, a preclinical gene therapy candidate for autosomal dominant Alzheimer's disease intended to silence the *APOE4* variant while expressing the a protective variant, and Novartis has a gene therapy candidate for Alzheimer's disease that is in the early preclinical stages of development. Many large and small pharmaceutical companies and academic institutions are developing potential treatments for the condition given the significant unmet need and the large population suffering from Alzheimer's disease. There are multiple FDA-approved treatments for Alzheimer's disease, including donepezil (Aricept), memantine (Namenda), and in January of 2023, lecanemab was granted accelerated approval by the FDA for the treatment of Alzheimer's disease based on the observed reduction of amyloid beta plaque and was granted full approval by the FDA in June 2023. In addition, Eli Lilly and Company's product candidate for the treatment of Alzheimer's disease, donanemab, has completed a Phase 3 clinical trial. Finally, we are aware that Voyager Therapeutics, Inc. is pursuing Alzheimer's disease treatments and have early-stage discovery efforts ongoing based on vectorized antibodies.

Many of our existing or potential competitors have substantially greater financial, technical and human resources than we do and significantly greater experience in the discovery and development of product candidates, as well as in obtaining regulatory approvals of those product candidates in the United States and in foreign countries. Our current and potential future competitors may also have significantly more experience commercializing drugs, particularly gene therapy and other biologics, that have been approved for marketing. Mergers and acquisitions in the pharmaceutical and biotechnology industries could result in even more resources being concentrated among a small number of our competitors.

We will face competition from other drugs or from other non-drug products currently approved or that will be approved in the future in the cardiac and neurology fields, including for the treatment of diseases and diseases in the therapeutic categories we intend to target. Therefore, our ability to compete successfully will depend largely on our ability to:

- develop and commercialize drugs that are advantageous as compared to other products in the market;
- demonstrate through our clinical trials that our product candidates are differentiated from existing and future therapies;
- attract qualified scientific, product development and commercial personnel;
- obtain patent or other proprietary protection for our product candidates;
- obtain required regulatory approvals;
- obtain coverage and adequate reimbursement from, and negotiate competitive pricing with, third-party payors; and
- successfully collaborate with other pharmaceutical companies in the discovery, development and commercialization of new medicines.

The availability of our competitors' products could limit the demand, and the price we are able to charge, for any product candidate we develop. The inability to compete with existing or subsequently introduced drugs

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would have an adverse impact on our business, financial condition and prospects. In addition, the reimbursement structure of approved gene therapies by other companies could impact the anticipated reimbursement structure of our gene therapies, if approved, and our business, financial condition, results of operations and prospects.

Established pharmaceutical companies may invest heavily to accelerate discovery and development of novel compounds or to in-license novel compounds that could make our product candidates less competitive. In addition, any new product that competes with an approved product must demonstrate compelling advantages in efficacy, convenience, tolerability and safety in order to overcome price competition and to be commercially successful. Accordingly, our competitors may succeed in obtaining patent protection, discovering, developing, receiving regulatory and marketing approval for, or commercializing, drugs before we do, which would have an adverse impact on our business and results of operations.

Any product candidates for which we intend to seek approval as biologic products may face competition sooner than anticipated.

The ACA includes a subtitle called the Biologics Price Competition and Innovation Act of 2009, or BPCIA, which created an abbreviated approval pathway for biologics that are biosimilar to or interchangeable with an FDA-licensed reference biological product. Under the BPCIA, an application for a biosimilar product may not be submitted to the FDA until four years following the date that the reference product was first licensed by the FDA. In addition, the approval of a biosimilar product may not be made effective by the FDA until 12 years from the date on which the reference product was first licensed. During this 12-year period of exclusivity, another company may still market a competing version of the reference product if the FDA approves a full BLA for the competing product containing the sponsor's own preclinical data and data from adequate and well-controlled clinical trials to demonstrate the safety, purity and potency of its product. The law is complex and is still being interpreted and implemented by the FDA. As a result, its ultimate impact, implementation and meaning are subject to uncertainty. While it is uncertain when such processes intended to implement BPCIA may be fully adopted by the FDA, any such processes could have an adverse effect on the future commercial prospects for our biologics.

There is a risk that any of our product candidates approved as a biological product under a BLA would not qualify for the 12-year period of exclusivity or that this exclusivity could be shortened due to congressional action or otherwise, or that the FDA will not consider our product candidates to be reference products for competing products, potentially creating the opportunity for generic competition sooner than anticipated. Other aspects of the BPCIA, some of which may impact the BPCIA exclusivity provisions, have also been the subject of recent litigation. Moreover, the extent to which a biosimilar, once approved, will be substituted for any one of our reference products in a way that is similar to traditional generic substitution for non-biologics is not yet clear, and will depend on a number of marketplace and regulatory factors that are still developing. If competitors are able to obtain marketing approval for biosimilars referencing our product candidates, if approved, our products may become subject to competition from such biosimilars, with the attendant competitive pressure and potential adverse consequences.

The success of our product candidates will depend significantly on coverage and adequate reimbursement or the willingness of patients, commercial and government payors to pay for these procedures.

We believe our success depends on obtaining and maintaining coverage and adequate reimbursement for our product candidates, including LX2006, LX1001 and LX2020, and the extent to which patients will be willing to pay out-of-pocket for such products, in the absence of reimbursement for all or part of the cost. In the United States and in other countries, patients who are provided medical treatment for their conditions generally rely on third-party payors to reimburse all or part of the costs associated with their treatment. The availability of coverage and adequacy of reimbursement for our products by third-party payors, including government health care programs (*e.g.*, Medicare, Medicaid, TRICARE), managed care providers, private health insurers, health maintenance organizations and other organizations is essential for most patients to be able to afford medical

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services and pharmaceutical products such as our product candidates. Third-party payors often rely upon Medicare coverage policy and payment limitations in setting their own coverage and reimbursement policies. However, decisions regarding the extent of coverage and amount of reimbursement to be provided are made on a payor-by-payor basis. One payor's determination to provide coverage for a drug product does not assure that other payors will also provide coverage, and adequate reimbursement. For more information, see the section titled "*Business — Government Regulation — Coverage and Reimbursement.*"

The principal decisions about reimbursement for new medicines are typically made by CMS, an agency within HHS. CMS decides whether and to what extent products will be covered and reimbursed under Medicare and private payors tend to follow CMS to a substantial degree.

Third-party payors determine which products and procedures they will cover and establish reimbursement levels. Even if a third-party payor covers a particular product or procedure, the resulting reimbursement payment rates may not be adequate. Patients who are treated in-office for a medical condition generally rely on third-party payors to reimburse all or part of the costs associated with the procedure, including costs associated with products used during the procedure, and may be unwilling to undergo such procedures in the absence of such coverage and adequate reimbursement. Physicians may be unlikely to offer procedures for such treatment if they are not covered by insurance and may be unlikely to purchase and use our product candidates, if approved, for our stated diseases unless coverage is provided and reimbursement is adequate. In addition, for products administered under the supervision of a physician, obtaining coverage and adequate reimbursement may be particularly difficult because of the higher prices often associated with such drugs. Further, coverage policies and third-party reimbursement rates may change at any time. Therefore, even if favorable coverage and reimbursement status is attained, less favorable coverage policies and reimbursement rates may be implemented in the future. In addition, companion diagnostic tests require coverage and reimbursement separate and apart from the coverage and reimbursement for their companion pharmaceutical or biological products. Similar challenges to obtaining coverage and reimbursement, applicable to pharmaceutical or biological products, will apply to companion diagnostics.

Reimbursement by a third-party payor may depend upon a number of factors, including the third-party payor's determination that a procedure is a covered benefit under its health plan; safe, effective and medically necessary; appropriate for the specific patient; cost-effective; supported by peer-reviewed medical journals; included in clinical practice guidelines; and neither cosmetic, experimental, nor investigational. Further, increasing efforts by third-party payors in the United States and abroad to cap or reduce healthcare costs may cause such organizations to limit both coverage and the level of reimbursement for newly approved products and, as a result, they may not cover or provide adequate payment for our product candidates. In order to secure coverage and reimbursement for any product that might be approved for sale, we may need to conduct expensive pharmacoeconomic studies in order to demonstrate the medical necessity and cost-effectiveness of our products, in addition to the costs required to obtain FDA or comparable regulatory approvals. Additionally, we may also need to provide discounts to purchasers, private health plans or government healthcare programs. Our product candidates may nonetheless not be considered medically necessary or cost-effective. If third-party payors do not consider a product to be cost-effective compared to other available therapies, they may not cover the product after approval as a benefit under their plans or, if they do, the level of payment may not be sufficient to allow a company to sell its products at a profit. We expect to experience pricing pressures from third-party payors in connection with the potential sale of any of our product candidates.

Foreign governments also have their own healthcare reimbursement systems, which vary significantly by country and region, and we cannot be sure that coverage and adequate reimbursement will be made available with respect to the treatments in which our products are used under any foreign reimbursement system.

There can be no assurance that LX2006, LX1001, LX2020 or any other product candidates, if approved for sale in the United States or in other countries, will be considered medically reasonable and necessary, that it will be considered cost-effective by third-party payors, that coverage or an adequate level of reimbursement will be

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available or that reimbursement policies and practices in the United States and in foreign countries where our products are sold will not adversely affect our ability to sell our product candidates profitably, if they are approved for sale.

Product liability lawsuits against us could cause us to incur substantial liabilities and to limit commercialization of any products that we may develop.

We face an inherent risk of product liability exposure related to the testing of our product candidates in human clinical trials and will face an even greater risk if we commercially sell any products that we may develop. If we cannot successfully defend ourselves against claims that our product candidates or drugs caused injuries, we will incur substantial liabilities. Regardless of merit or eventual outcome, liability claims may result in:

- decreased demand for any product candidates or drugs that we may develop;
- injury to our reputation and significant negative media attention;
- withdrawal of clinical trial participants;
- significant costs to defend the related litigation;
- substantial monetary awards paid to trial participants or patients;
- loss of revenue;
- reduced resources of our management to pursue our business strategy; and
- the inability to commercialize any products that we may develop.

Although we maintain product liability insurance coverage, such insurance may not be adequate to cover all liabilities that we may incur. We may need to increase our insurance coverage as we expand our clinical trials or if we commence commercialization of our product candidates. Insurance coverage is increasingly expensive. We may not be able to maintain insurance coverage at a reasonable cost or in an amount adequate to satisfy any liability that may arise.

Risks related to our dependence on third parties

Currently, we rely on our collaborations with Cornell University and UCSD to conduct research and development for many of our pipeline programs, including conducting preclinical and IND-enabling studies for portions of our near-term future pipeline. Failure or delay of Cornell University or UCSD to fulfill all or part of their respective obligations to us under our agreements, a breakdown in collaboration between the parties or a complete or partial loss of either of these relationships could materially harm our business.

Our collaboration with Cornell University is critical to our business and in May 2020, we entered into two separate license agreements with Cornell University for preclinical research and development collaborations and non-exclusive license rights to patents for certain products and technologies. As part of our first license agreement, as amended, we assumed oversight for the conduct of the Phase 1/2 clinical trial of LX1001 that was initiated by Cornell University at the end of 2019. Pursuant to these license agreements, we are obligated to diligently proceed with the development, manufacture, and sale of licensed products. If Cornell University delays or fails to perform its obligations under the license agreements, disagrees with our interpretation of their terms, or terminates any of the license agreements, our pipeline of product candidates would be significantly adversely affected and our prospects may be materially harmed.

Our collaboration with UCSD is also highly important to our business, as we have licensed from UCSD intellectual property rights related to our LX2020, LX2021 and LX2022 programs under three separate license agreements, and we have entered into sponsored research agreements with UCSD for preclinical research and

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development for these programs. If UCSD delays or fails to perform its obligations under either of the sponsored research agreements, disagrees with our interpretation of the terms of the sponsored research agreement or our discovery plan or terminates any of our existing license agreements, our pipeline of product candidates would be significantly adversely affected and our prospects may be materially harmed.

We intend to continue to rely on third parties to conduct a significant portion of our existing clinical trials and potential future clinical trials for product candidates, and those third parties may not perform satisfactorily, including failing to meet deadlines for the completion of such trials.

We engage CROs to help conduct our ongoing clinical trials. We expect to continue to rely on third parties, including clinical data management organizations, medical institutions and clinical investigators, to conduct those clinical trials and any future clinical trials. Any of these third parties may terminate their engagements with us, some in the event of an uncured material breach and some at any time for convenience. If any of our relationships with these third parties terminate, we may not be able to timely enter into arrangements with alternative third parties or to do so on commercially reasonable terms, if at all. Switching or adding a CRO involves substantial cost and requires management time and focus. In addition, there is a natural transition period when a new CRO commences work. As a result, delays occur, which can materially impact our ability to meet our desired clinical development timelines. Though we intend to carefully manage our relationships with our CROs, there can be no assurance that we will not encounter challenges or delays in the future or that these delays or challenges will not have a material adverse impact on our business, financial condition and prospects.

In addition, any third parties conducting our clinical trials will not be our employees, and except for remedies available to us under our agreements with such third parties, we cannot control whether or not they devote sufficient time and resources to our clinical programs. If these third parties do not successfully carry out their contractual duties or obligations or meet expected deadlines, if they need to be replaced or if the quality or accuracy of the clinical data they obtain is compromised due to the failure to adhere to our clinical protocols, regulatory requirements or for other reasons, our clinical trials may be extended, delayed or terminated and we may not be able to obtain regulatory approval for or successfully commercialize our product candidates. Consequently, our results of operations and the commercial prospects for our product candidates would be harmed, our costs could increase substantially and our ability to generate revenue could be delayed significantly.

We rely on these parties for execution of our preclinical studies and clinical trials, and generally do not control their activities. Our reliance on these third parties for research and development activities will reduce our control over these activities but will not relieve us of our responsibilities. For example, we will remain responsible for ensuring that each of our clinical trials is conducted in accordance with the general investigational plan and protocols for the trial. Moreover, the FDA requires us to comply with cGCP regulations, for conducting, recording and reporting the results of clinical trials to assure that data and reported results are credible and accurate and that the rights, integrity and confidentiality of trial participants are protected. We also are required to register ongoing clinical trials and post the results of completed clinical trials on a government-sponsored database, www.clinicaltrials.gov, within specified time frames. Failure to do so can result in fines, adverse publicity and civil and criminal sanctions. If we or any of our CROs or other third parties, including trial sites, fail to comply with applicable cGCPs, or experience material protocol deviations, the clinical data generated in our clinical trials may be deemed unreliable and the FDA, EMA or comparable foreign regulatory authorities may require us to perform additional clinical trials before approving our marketing applications. If FDA or any regulatory authority determines that our clinical data are not reliable for any reason, or if we encounter any data integrity issues, FDA or other regulatory authorities may require us to exclude such data, which may cause the trial to be underpowered and fail to meet the trial endpoints. We cannot assure you that upon inspection by a given regulatory authority, such regulatory authority will determine that any of our clinical trials complies with cGCP regulations. In addition, our clinical trials must be conducted with product produced under cGMP conditions. Our failure to comply with these regulations may require us to repeat clinical trials, which would delay the regulatory approval process.

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In addition, principal investigators for our clinical trials may serve as scientific advisors or consultants to us from time to time and receive compensation in connection with such services. Under certain circumstances, we may be required to report some of these relationships to the FDA. The FDA may conclude that a financial relationship between us and a principal investigator has created a conflict of interest or otherwise affected interpretation of the trial. The FDA may therefore question the integrity of the data generated at the applicable clinical trial site and the utility of the clinical trial itself may be jeopardized. This could result in a delay in approval, or rejection, of our marketing applications by the FDA and may ultimately lead to the denial of marketing approval of LX2006, LX1001, LX2020 or any other product candidates.

We also expect to rely on other third parties to store and distribute product supplies for our clinical trials. Any performance failure on the part of our distributors could delay clinical development or marketing approval of our product candidates or commercialization of our products, producing additional losses and depriving us of potential revenue.

We may seek collaborations with non-academic third parties for the development or commercialization of our product candidates. If those collaborations are not successful, we may not be able to capitalize on the market potential of these product candidates.

We may seek third-party collaborators for the development and commercialization of our product candidates, including for the commercialization of any of our product candidates that are approved for marketing outside the United States. Our likely collaborators for any such arrangements include regional and national pharmaceutical companies and biotechnology companies. If we enter into any additional such arrangements with any third parties, we will likely have limited control over the amount and timing of resources that our collaborators dedicate to the development or commercialization of our product candidates. Our ability to generate revenue from these arrangements will depend on our collaborators' abilities to successfully perform the functions assigned to them in these arrangements. Collaborations involving our product candidates pose the following risks to us:

- collaborators have significant discretion in determining the efforts and resources that they will apply to these collaborations;
- collaborators may not perform their obligations as expected;
- collaborators may not pursue development and commercialization of any product candidates that achieve regulatory approval or may elect not to continue or renew development or commercialization programs based on clinical trial results, changes in the collaborators' strategic focus or available funding, or external factors, such as an acquisition, that divert resources or create competing priorities;
- collaborators may delay clinical trials, provide insufficient funding for a clinical trial program, stop a clinical trial or abandon a product candidate, repeat or conduct new clinical trials or require a new formulation of a product candidate for clinical testing;
- collaborators could independently develop, or develop with third parties, products that compete directly or indirectly with our product candidates if the collaborators believe that competitive products are more likely to be successfully developed or can be commercialized under terms that are more economically attractive than ours;
- we could grant exclusive rights to our collaborators that would prevent us from collaborating with others;
- product candidates discovered in collaboration with us may be viewed by our collaborators as competitive with their own product candidates or drugs, which may cause collaborators to cease to devote resources to the commercialization of our product candidates;
- a collaborator with marketing and distribution rights to one or more of our product candidates that achieve regulatory approval may not commit sufficient resources to the marketing and distribution of such products;

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- disagreements with collaborators, including disagreements over proprietary rights, contract interpretation or the preferred course of development, might cause delays or termination of the research, development or commercialization of product candidates, might lead to additional responsibilities for us with respect to product candidates, or might result in litigation or arbitration, any of which would be time-consuming and expensive;
- collaborators may not properly maintain or defend our or their intellectual property rights or may use our or their proprietary information in such a way as to invite litigation that could jeopardize or invalidate such intellectual property or proprietary information or expose us to potential litigation;
- collaborators may infringe the intellectual property rights of third parties, which may expose us to litigation and potential liability; and
- collaborations may be terminated for the convenience of the collaborator and, if terminated, we could be required to raise additional capital to pursue further development or commercialization of the applicable product candidates.

Collaboration agreements may not lead to development or commercialization of product candidates in the most efficient manner or at all. If any future collaborator of ours were to be involved in a business combination, the continued pursuit and emphasis on our product development or commercialization program could be delayed, diminished or terminated.

We face significant competition in seeking appropriate collaborators. Whether we reach a definitive agreement for any collaboration will depend, among other things, upon our assessment of the collaborator's resources and expertise, the terms and conditions of the proposed collaboration and the proposed collaborator's evaluation of a number of factors. Those factors may include the design or results of clinical trials, the likelihood of approval by the FDA or similar regulatory authorities outside the United States, the potential market for the subject product candidate, the costs and complexities of manufacturing and delivering such product candidate to patients, the potential of competing products, the existence of uncertainty with respect to our ownership of technology, which can exist if there is a challenge to such ownership without regard to the merits of the challenge and industry and market conditions generally. The collaborator may also consider alternative product candidates or technologies for similar diseases that may be available to collaborate on and whether such a collaboration could be more attractive than the one with us for our product candidate. Collaborations are complex and time-consuming to negotiate and document. In addition, there have been a significant number of recent business combinations among large pharmaceutical companies that have resulted in a reduced number of potential future collaborators.

We may not be able to negotiate additional collaborations on a timely basis, on acceptable terms, or at all. If we are unable to do so, we may have to curtail the development of such product candidate, reduce or delay its development program or one or more of our other development programs, delay its potential commercialization or reduce the scope of any sales or marketing activities, or increase our expenditures and undertake development or commercialization activities at our own expense. If we elect to increase our expenditures to fund development or commercialization activities on our own, we may need to obtain additional capital, which may not be available to us on acceptable terms or at all. If we do not have sufficient funds, we may not be able to further develop our product candidates or bring them to market and generate revenue.

Risks related to intellectual property

If we are unable to obtain or protect intellectual property rights related to any of our product candidates, we may not be able to compete effectively in our market.

We rely upon a combination of patents, trade secret protection and confidentiality agreements to protect the intellectual property related to our product candidates, including LX2006, LX2020, LX2021, LX2022, LX1001, LX1020, LX1021, and other programs, their respective components, formulations, therapies, methods used to

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manufacture them and methods treatment. Furthermore, we currently do not have any patents or patent applications covering our LX1004 product candidate.

Our success depends in large part on our ability to obtain and maintain patent and other intellectual property protection in the United States and in other countries with respect to our proprietary technology and product candidates.

We cannot offer any assurances about which of our patent applications will issue, the breadth of any resulting patent or whether any of the issued patents will be found invalid and unenforceable or will be threatened by third parties. We cannot offer any assurances that the breadth of our granted patents will be sufficient to stop a competitor from developing and commercializing a product, including a biosimilar product that would be competitive with one or more of our product candidates. Furthermore, any successful challenge to these patents or any other patents owned by or licensed to us after patent issuance could deprive us of rights necessary for the successful commercialization of any of our product candidates. Further, if we encounter delays in regulatory approvals, the period of time during which we could market a product candidate under patent protection could be reduced.

The patent prosecution process is expensive and time-consuming. We may not be able to prepare, file and prosecute all necessary or desirable patent applications at a commercially reasonable cost or in a timely manner or in all jurisdictions. It is also possible that we may fail to identify patentable aspects of inventions made in the course of development and commercialization activities before it is too late to obtain patent protection on them. We may not be able to obtain or maintain patent applications and patents due to the subject matter claimed in such patent applications and patents being in the public domain. In some cases, the work of certain academic researchers in the gene therapy field has entered the public domain, which may preclude our ability to obtain patent protection for certain inventions relating to such work. Although we enter into nondisclosure and confidentiality agreements with parties who have access to confidential or patentable aspects of our research and development output, such as our employees, corporate collaborators, outside scientific collaborators, contract manufacturers, consultants, advisors and other third parties, any of these parties may breach these agreements and disclose such output before a patent application is filed, thereby jeopardizing our ability to seek patent protection. Consequently, we would not be able to prevent any third party from using any technology that is in the public domain to compete with our product candidates. Moreover, depending on the terms of any future in-licenses to which we may become a party, we may not have the right to control the preparation, filing and prosecution of patent applications, or to maintain the patents, covering technology in-licensed from third parties. Therefore, these patents and patent applications may not be prosecuted and enforced in a manner consistent with the best interests of our business.

The patent position of biotechnology and pharmaceutical companies generally is highly uncertain, involves complex legal and factual questions and has, in recent years, been the subject of much litigation. As a result, the issuance, scope, validity, enforceability and commercial value of any patent rights are highly uncertain. Our owned and licensed pending and future patent applications may not result in issued patents which protect our technology or product candidates, effectively prevent others from commercializing competitive technologies and product candidates or otherwise provide any competitive advantage. In fact, patent applications may not issue as patents at all. Even if patent applications we license or own currently or in the future issue as patents, they may not issue in a form that will provide us with any meaningful protection, prevent competitors or other third parties from competing with us, or otherwise provide us with any competitive advantage. Any patents that we hold or in-license may be challenged, narrowed, circumvented, or invalidated by third parties. Consequently, we do not know whether any of our technologies and product candidates will be protectable or remain protected by valid and enforceable patents. In addition, our existing patents and any future patents we obtain may not be sufficiently broad to prevent others from using our technology or from developing competing technologies and products, and the coverage claimed in a patent application can be significantly reduced before the patent is issued, and its scope can be reinterpreted after issuance. Any failure to obtain, maintain or defend our patents and other intellectual property could have a material adverse effect on our business, financial conditions, results of operations and prospects.

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We cannot be certain that we are the first to invent the inventions covered by pending patent applications and, if we are not, we may be subject to priority or entitlement disputes. We may be required to disclaim part or all of the term of certain patents or all of the term of certain patent applications. There may be prior art of which we are not aware that may affect the validity or enforceability of a patent claim. There also may be prior art of which we are aware, but which we do not believe affects the validity or enforceability of a claim, which may, nonetheless, ultimately be found to affect the validity or enforceability of a claim. Since patent applications in the United States and other countries are confidential for a period of time after filing, at any moment in time, we cannot be certain that we were in the past or will be in the future the first to file any patent application related to our product candidates. For example, some patent applications in the United States may be maintained in secrecy until the patents are issued. Further, publications in the scientific literature often lag behind actual discoveries. Consequently, we cannot be certain that others have not filed patent applications for technology covered by our owned and in-licensed issued patents or our pending applications, or that we or, if applicable, a licensor, were the first to invent or first to file an application for the technology.

It is possible that defects of form in the preparation or filing of our patents or patent applications may exist, or may arise in the future, for example, with respect to proper priority claims, inventorship, claim scope, or requests for patent term adjustments. If there are material defects in the form, preparation, prosecution, or enforcement of our patents or patent applications, such patents may be invalid and/or unenforceable, and such applications may never result in valid, enforceable patents. Any of these outcomes could impair our ability to prevent competition from third parties, which may have an adverse impact on our business.

In addition to the protection provided by our patent estate, we rely on trade secret protection and confidentiality agreements to protect proprietary know-how that is not amenable to patent protection. Although we generally require all of our employees to assign their inventions to us, and all of our employees, consultants, advisors and any third parties who have access to our proprietary know-how, information, or technology to enter into confidentiality agreements, we cannot provide any assurances that all such agreements have been duly executed, or that our trade secrets and other confidential proprietary information will not be disclosed. In addition, while we have undertaken reasonable efforts to ensure such agreements are enforceable and that employees and third parties comply with their obligations thereunder, these agreements may be found insufficient by a court of law or may be breached, or we may not enter into sufficient agreements with such individuals in the first instance, in either case potentially resulting in the unauthorized use or disclosure of our trade secrets and other intellectual property, including to our competitors, which could cause us to lose any competitive advantage resulting from this intellectual property. Individuals not subject to invention assignment agreements may make adverse ownership claims to our current and future intellectual property. Moreover, our competitors may independently develop knowledge, methods and know-how equivalent to our trade secrets. Competitors could purchase our products, if approved, and replicate some or all of the competitive advantages we derive from our development efforts for technologies on which we do not have patent protection. If any of our trade secrets were to be lawfully obtained or independently developed by a competitor, we would have no right to prevent them, or those to whom they communicate it, from using that technology or information to compete with us. If any of our trade secrets were to be disclosed to or independently developed by a competitor, our competitive position would be harmed. Enforcing a claim that a third-party entity illegally obtained and is using any of our trade secrets is expensive and time-consuming, and the outcome is unpredictable, and we may not be able to obtain adequate remedies for such breaches.

We also seek to preserve the integrity and confidentiality of our data and trade secrets by maintaining physical security of our premises and physical and electronic security of our information technology systems. While we have confidence in these individuals, organizations and systems, our agreements or security measures may be breached, and we may not have adequate remedies for any breach. Also, if the steps taken to maintain our trade secrets are deemed inadequate, we may have insufficient recourse against third parties for misappropriating the trade secret. In addition, others may independently discover our trade secrets and proprietary information. For example, the FDA is considering whether to make additional information publicly available on a routine basis, including information that we may consider to be trade secrets or other proprietary information, and it is not clear

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at the present time how the FDA's disclosure policies may change in the future. If we are unable to prevent material disclosure of the non-patented intellectual property related to our technologies to third parties, and there is no guarantee that we will have any such enforceable trade secret protection, we may not be able to establish or maintain a competitive advantage in our market, which could materially adversely affect our business, results of operations and financial condition.

Patent terms may be inadequate to protect our competitive position on our products for an adequate amount of time, and if we do not obtain protection under the Hatch-Waxman Amendments and similar non-United States legislation for extending the term of patents covering each of our product candidates, our business may be materially harmed.

Given the amount of time required for the development, testing and regulatory review of new product candidates, patents protecting such candidates might expire before or shortly after such product candidates are commercialized. Depending upon the timing, duration and conditions of FDA marketing approval of our product candidates, one or more of our United States patents may be eligible for limited patent term extension under the Drug Price Competition and Patent Term Restoration Act of 1984, referred to as the Hatch-Waxman Amendments, and similar legislation in the European Union. The Hatch-Waxman Amendments permit a patent term extension of up to five years for a patent covering an approved product as compensation for effective patent term lost during product development and the FDA regulatory review process. A patent term extension cannot extend the remaining term of a patent beyond a total of 14 years from the date of product approval. Only one patent may be extended, and only those claims covering the approved drug, a method for using it, or a method for manufacturing it may be extended. However, we may not receive an extension if we fail to apply within applicable deadlines, fail to apply prior to expiration of relevant patents or otherwise fail to satisfy applicable requirements. Moreover, the length of the extension could be less than we request. If we are unable to obtain patent term extension or the term of any such extension is less than we request, the period during which we can enforce our patent rights for that product will be shortened and our competitors may obtain approval to market competing products sooner. As a result, our revenue from applicable products could be reduced and could have a material adverse effect on our business.

We in-license key intellectual property necessary for the development of each of our current product candidates. If we fail to comply with our obligations in our current and future intellectual property licenses with third parties, resulting in the termination of such licenses, we could lose rights that are important to our business.

We are heavily reliant upon licenses to certain patent rights and proprietary technology for the development of each of our current product candidates. In particular, we in-license key patents and patent applications from Adverum related to LX2006, we in-license patent applications and know-how from Cornell University related to our LX1001, LX1020 and LX1021 product candidates, and we in-license patent applications and know-how from the Regents of the University of California, San Diego, related to our LX2020, LX2021 and LX2022 product candidates. Our license agreements impose diligence and milestone and royalty payment obligations on us, and also contain certain development requirements. If we fail to comply with our obligations, our licensors may have the right to terminate our licenses, in which event we will not be able to develop, manufacture or market any product using the intellectual property under any such terminated agreement and may face other penalties. Such an occurrence would materially adversely affect our business prospects.

Certain of our licenses may not provide us with exclusive rights to use the licensed intellectual property and technology, or may not provide us with exclusive rights to use such intellectual property and technology in all relevant fields of use and in all territories in which we may wish to develop or commercialize our technology and product candidates in the future. In addition, the intellectual property rights licensed to us by our licensors, including certain intellectual property licensed by Cornell University, The Regents of the University of California, San Diego, and Adverum, at least in some respects, may be used by such licensors or licensed to third parties, and such third parties may have certain enforcement rights with respect to such intellectual property.

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Thus, patents licensed to us could be put at risk of being invalidated or interpreted narrowly in litigation filed by or against our licensors or another licensee or in administrative proceedings brought by or against our licensors or another licensee in response to such litigation or for other reasons. As a result, we may not be able to prevent competitors or other third parties from developing and commercializing competitive products, including in territories covered by our licenses.

Licenses to additional third-party technology and materials that may be required for our development programs may not be available in the future or may not be available on commercially reasonable terms, or at all, which could have a material adverse effect on our business and financial condition. In such events, we may be required to expend significant time and resources to redesign our technology, product candidates, or the methods for manufacturing them or to develop or license replacement technology, all of which may not be feasible on a technical or commercial basis. If we are unable to do so, we may be unable to develop or commercialize the affected technology or product candidates. Even if we are able to obtain such additional licenses, they may be non-exclusive thereby giving our competitors and other third parties access to the same technology licensed to us.

If we or our licensors fail to adequately protect our licensed intellectual property, our ability to commercialize our product candidates and technology could suffer. Although we have oversight rights, Cornell University and The Regents of the University of California, San Diego, generally control the prosecution, maintenance and enforcement of our in-licensed patents and patent applications. Therefore, we cannot be certain that the prosecution, maintenance and enforcement of these patent rights will be in a manner consistent with the best interests of our business, or in compliance with applicable laws and regulations, or will result in valid and enforceable patents and other intellectual property rights. It is possible that our licensors' infringement proceedings or defense activities may be less vigorous than had we conducted them ourselves or may not be conducted in accordance with our best interests. If we or our licensors fail to maintain such patents or patent applications, or if we or our licensor lose rights to those patents or patent applications, the rights we have licensed may be reduced or eliminated and our right to develop and commercialize any of our product candidates that are the subject of such licensed rights could be adversely affected. In addition to the foregoing, the risks associated with patent rights that we license from third parties will also apply to patent rights we may own in the future.

Further, if we fail to comply with our development obligations under our license agreements, we may lose our patent rights with respect to such agreement on a territory-by-territory basis, which would affect our patent rights worldwide. In spite of our efforts, our current and future licensors might conclude that we have materially breached our obligations under our license agreements and might therefore terminate such license agreements, thereby removing or limiting our ability to develop and commercialize products and technology covered by these license agreements. Disputes may also arise between us and our licensors regarding intellectual property subject to a license agreement, including:

- the scope of rights granted under the license agreement and other interpretation-related issues;
 - our financial and other obligations under the license agreement;
 - whether and the extent to which our technology and processes infringe on intellectual property of the licensor that is not subject to the licensing agreement;
 - our right to sublicense patent and other rights to third parties under collaborative development relationships;
 - our diligence obligations with respect to the use of the licensed technology in relation to our development and commercialization of our product candidates and what activities satisfy those diligence obligations;
 - the inventorship or ownership of inventions and know-how resulting from the joint creation or use of intellectual property by our licensors and us and our partners; and
- the priority of invention of patented technology.

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If disputes over intellectual property that we have licensed prevent or impair our ability to maintain our current licensing arrangements on acceptable terms, we may be unable to successfully develop and commercialize the affected technology or product candidates. In addition, if any such disputes result in the termination of our intellectual property licenses, this could result in the loss of our ability to develop and commercialize our lead product candidates, or we could lose other significant rights, experience significant delays in the development and commercialization of our other product candidates, or incur liability for damages, any of which could have a material adverse effect on our business, financial condition, results of operations and prospects. In addition, we may seek to obtain additional licenses from our licensors and, in connection with obtaining such licenses, we may agree to amend our existing licenses in a manner that may be more favorable to the licensors, including by agreeing to terms that could enable third parties, including our competitors, to receive licenses to a portion of the intellectual property that is subject to our existing licenses and to compete with our product candidates.

Some of our future agreements with certain of our third-party research partners may provide that improvements developed in the course of our relationship may be owned solely by either us or our third-party research partner. If we determine that rights to such improvements owned solely by a third-party research partner or other third party with whom we collaborate are necessary to commercialize our therapeutic product candidates or maintain our competitive advantage, we may need to obtain a license from such third party in order to use the improvements and continue developing, manufacturing or marketing our drug candidates. We may not be able to obtain such a license on an exclusive basis, on commercially reasonable terms, or at all, which could prevent us from commercializing our product candidates or allow our competitors or others the chance to access technology that is important to our business.

Termination of our current or any future license agreements would reduce or eliminate our rights under these agreements and may result in our having to negotiate new or reinstated agreements with less favorable terms or cause us to lose our rights under these agreements, including our rights to important intellectual property or technology. Any of the foregoing could prevent us from commercializing our other product candidates, which could have a material adverse effect on our operating results and overall financial condition.

In addition, intellectual property rights that we in-license in the future may be sublicenses under intellectual property owned by third parties, in some cases through multiple tiers. The actions of our licensors may therefore affect our rights to use our sublicensed intellectual property, even if we are in compliance with all of the obligations under our license agreements. Should our licensors or any of the upstream licensors fail to comply with their obligations under the agreements pursuant to which they obtain the rights that are sublicensed to us, or should such agreements be terminated or amended, our ability to develop and commercialize our product candidates may be materially harmed.

In addition, a third party may in the future bring claims that our performance under our license agreements, including our sponsoring of clinical trials, interferes with such third party's rights under its agreement with one of our licensors. If any such claim were successful, it may adversely affect our rights and ability to advance our product candidates as clinical candidates or subject us to liability for monetary damages, any of which would have an adverse effect on our business, financial condition, results of operations and prospects.

We are generally also subject to all of the same risks with respect to protection of intellectual property that we license as we are for intellectual property that we own, which are described above and below. If we or our licensors fail to adequately protect this intellectual property, our ability to commercialize products could suffer. We may not be successful in obtaining or maintaining necessary rights to product components and processes for our development pipeline through acquisitions and in-licenses.

Presently, we have obtained rights to certain intellectual property rights through licenses from third parties to develop, manufacture and commercialize our lead product candidates and other potential product candidates in

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our pipeline. Because the commercialization of our product candidates may require the use of additional intellectual property rights held by third parties, the growth of our business likely will depend, in part, on our ability to acquire or license these intellectual property rights. Our product candidates also require specific formulations and manufacturing processes to work effectively and efficiently, and some of these rights are held by others.

We may be unable to acquire or in-license any compositions, methods of use, processes or other third-party intellectual property rights from third parties that we identify as necessary, important or more expedient to further our business operations. In addition, even if we are able to obtain such licenses, we may fail to obtain any of these licenses at a reasonable cost or on reasonable terms, which would harm our business. Were that to happen, we may need to cease use of the product candidates and technologies covered by those third-party intellectual property rights and may need to seek to develop alternative approaches that do not infringe, misappropriate or violate those intellectual property rights, which may entail additional costs and development delays if we are able to develop such alternatives, or which may not be feasible. Even if we are able to obtain a license, it may be non-exclusive, which means that our competitors may also receive access to the same technologies licensed to us. The licensing and acquisition of third-party intellectual property rights is a competitive practice, and companies that may be more established, or have greater resources than we do, may also be pursuing strategies to license or acquire third-party intellectual property rights that we may consider necessary or attractive in order to commercialize our product candidates. More established companies may have a competitive advantage over us due to their larger size and cash resources or greater clinical development and commercialization capabilities. In addition, companies that perceive us to be a competitor may be unwilling to assign or license rights to us. There can be no assurance that we will be able to successfully complete such negotiations and ultimately acquire the rights to the intellectual property surrounding the additional product candidates that we may seek to acquire. We also may be unable to license or acquire third-party intellectual property rights on terms that would allow us to make an appropriate return on our investment.

If we are unable to successfully obtain rights to required third-party intellectual property or maintain the existing intellectual property rights we have licensed, we may be required to expend significant time and resources to redesign our product candidates, or to develop or license replacement technology, all of which may not be feasible on a technical or commercial basis, and we may have to abandon development of our product candidates, any of which could have a material adverse effect on our business, financial condition, results of operations and prospects.

Our intellectual property licenses with third parties may be subject to disagreements over contract interpretation, which could narrow the scope of our rights to the relevant intellectual property or technology or increase our financial or other obligations to our licensors.

We currently depend, and will continue to depend, on our license agreements, including the license agreements with Cornell University and Adverum related to LX2006, with Cornell University related to our LX1001, LX1020 and LX1021 product candidates, and with The Regents of the University of California, San Diego, related to LX2020, LX2021 and LX2022 product candidates. The agreements under which we currently license intellectual property or technology from third parties are complex, and certain provisions in such agreements may be susceptible to multiple interpretations. The resolution of any contract interpretation disagreement that may arise could narrow what we believe to be the scope of our rights to the relevant intellectual property or technology, or increase what we believe to be our financial or other obligations under the relevant agreement, either of which could have a material adverse effect on our business, financial condition, results of operations and prospects.

If any of our licenses or material relationships or any in-licenses upon which our licenses are based are terminated or breached, we may:

- lose our rights to develop and market our products;

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- lose patent protection for our products;
- experience significant delays in the development or commercialization of our products;
- not be able to obtain any other licenses on acceptable terms, if at all; or
- incur liability for damages.

These risks apply to any agreements that we may enter into in the future for our products or for any future product candidates. If we experience any of the foregoing, it could have a material adverse effect on our business, financial condition, results or operations and prospects.

We cannot be certain that any of our or licensed pending patent applications or our future owned or licensed patent applications will result in issued patent claims covering such aspects of our product candidates.

Composition-of-matter patents on the active pharmaceutical ingredient, or API, in prescription drug products are generally considered to be the strongest form of intellectual property protection for drug products because those types of patents provide protection without regard to any particular method of use or manufacture or formulation of the API used. Although we intend to file patent applications in the future that cover these product candidates, we cannot be certain that our future owned or licensed patent applications will cover our current or future product candidates.

Method-of-use patents protect the use of a product for the specified method and formulation patents cover formulations of the API. These types of patents do not prevent a competitor or other third party from developing or marketing an identical product for an indication that is outside the scope of the patented method or from developing a different formulation that is outside the scope of the patented formulation. Moreover, with respect to method-of-use patents, even if competitors or other third parties do not actively promote their product for our targeted indications or uses for which we may obtain patents, physicians may recommend that patients use these products off-label, or patients may do so themselves. Although off-label use may infringe or contribute to the infringement of method-of-use patents, the practice is common, and this type of infringement is difficult to prevent or prosecute. In addition, there are numerous publications and other prior art that may be relevant to our owned or in-licensed method-of-use patents and patent applications and may be used to challenge the validity of these owned or in-licensed patents and patent applications in litigation or other intellectual property-related proceedings. If these types of challenges are successful, our owned or in-licensed patents and patent applications may be narrowed or found to be invalid, and we may lose valuable intellectual property rights. Any of the foregoing could have a material adverse effect on our business, financial conditions, prospects and results of operations.

The strength of patents in the biotechnology and pharmaceutical field involves complex legal and scientific questions and can be uncertain. The patent applications that we own or in-license may fail to result in issued patents with claims that cover our product candidates or uses thereof in the United States or in other countries. Even if patents do successfully issue, the issuance of a patent is not conclusive as to its inventorship, scope, validity or enforceability and third parties may challenge the validity, enforceability or scope of our owned and licensed patents in courts or patent offices in the United States and abroad, which may result in those patents being narrowed, invalidated or held unenforceable. Furthermore, even if they are unchallenged, our owned and licensed patents and pending patent applications, if issued, may not adequately protect our intellectual property or prevent competitors or others from designing around our patent claims to circumvent our owned or licensed patents by developing similar or alternative technologies or therapeutics in a non-infringing manner. If the breadth or strength of protection provided by the patents and patent applications we own or license with respect to our product candidates is not sufficient to impede such competition or is otherwise threatened, it could dissuade companies from collaborating with us to develop, and threaten our ability to commercialize, our product candidates. Any of the foregoing could have a material adverse effect on our business, financial condition, results of operations and prospects.

We may be involved in lawsuits to protect or enforce our patents, which could be expensive, time-consuming and unsuccessful.

Competitors may infringe the patents for which we have applied. To counter infringement or unauthorized use, we may be required to file infringement claims, which can be expensive and time-consuming. If we initiate legal proceedings against a third party to enforce a patent covering one of our product candidates, the defendant could counterclaim that the patent covering our product or product candidate is invalid and/or unenforceable. In patent litigation in the United States, counterclaims alleging invalidity and/or unenforceability are common, and there are numerous grounds upon which a third party can assert invalidity or unenforceability of a patent. In an infringement proceeding, a court may decide that the patent claims we are asserting are invalid and/or unenforceable, or may refuse to stop the other party from using the technology at issue on the grounds that our patent claims do not cover the technology in question. Third parties may also raise similar claims before administrative bodies in the United States or abroad, even outside the context of litigation. Such mechanisms include re-examination, post-grant review, inter partes review and equivalent proceedings in foreign jurisdictions (for example, opposition proceedings). Such proceedings could result in revocation of or amendment to our patents in such a way that they no longer cover our product candidates. The outcome following legal assertions of invalidity and unenforceability is unpredictable. With respect to the validity question, for example, we cannot be certain that there is no invalidating prior art, of which we, our patent counsel and the patent examiner were unaware during prosecution. If a defendant were to prevail on a legal assertion of invalidity and/or unenforceability, we would lose at least part, and perhaps all, of the patent protection on our product candidates. An adverse result in any litigation or defense proceedings could put one or more of our patents at risk of being invalidated or interpreted narrowly, could put our patent applications at risk of not issuing and could have a material adverse impact on our business.

Interference proceedings provoked by third parties or brought by us may be necessary to determine the priority of inventions with respect to our patent applications. An unfavorable outcome could require us to cease using the related technology or force us to take a license under the patent rights of the prevailing party, if available. Furthermore, our business could be harmed if the prevailing party does not offer us a license on commercially reasonable terms. Our defense of litigation or interference proceedings may fail and, even if successful, may result in substantial costs and distract our management and other employees. We may not be able to prevent misappropriation of our intellectual property rights, particularly in countries where the laws may not protect those rights as fully as in the United States.

Even if we establish infringement of any of our patents by a competitive product, a court may decide not to grant an injunction against further infringing activity, thus allowing the competitive product to continue to be marketed by the competitor. It is difficult to obtain an injunction in U.S. litigation and a court could decide that the competitor should instead pay us a “reasonable royalty” as determined by the court, and/or other monetary damages. A reasonable royalty or other monetary damages may or may not be an adequate remedy. Loss of exclusivity and/or competition from a related product would have a material adverse impact on our business.

For certain of our in-licensed patent rights, such as patent rights in-licensed from Cornell University and Adverum, we may not have the right to file a lawsuit for infringement and may have to rely on a licensor to enforce these rights for us. If we are not able to directly assert our licensed patent rights against infringers or if a licensor does not vigorously prosecute any infringement claims on our behalf, we may have difficulty competing in certain markets where such potential infringers conduct their business, and our commercialization efforts may suffer as a result.

In addition, we or our licensors, as the case may be, may not be able to detect infringement against our owned or in-licensed patents, which may be especially difficult for manufacturing processes or formulation patents. Even if we or our licensors detect infringement by a third party of our owned or in-licensed patents, we or our licensors, as the case may be, may choose not to pursue litigation against or settlement with the third party. If we or our licensors later sue such third party for patent infringement, the third party may have certain legal

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defenses available to it that otherwise would not be available but for the delay between when the infringement was first detected and when the suit was brought. These legal defenses may make it impossible for us or our licensors to enforce our owned or in-licensed patents, as the case may be, against that third party.

Furthermore, because of the substantial amount of discovery required in connection with intellectual property litigation, there is a risk that some of our confidential information could be compromised by disclosure during this type of litigation. There could also be public announcements of the results of hearings, motions, or other interim proceedings or developments. If securities analysts or investors perceive these results to be negative, it could have a material adverse effect on the price of our Common Stock.

Third parties may initiate legal proceedings alleging that we are infringing their intellectual property rights, the outcome of which would be uncertain.

As our current and future product candidates progress toward commercialization, the possibility of a patent infringement claim against us increases. We cannot provide any assurance that our current and future product candidates do not infringe other parties' patents or other proprietary rights, and competitors or other parties may assert that we infringe their proprietary rights in any event. We may become party to, or threatened with, adversarial proceedings or litigation regarding intellectual property rights with respect to our current and future product candidates, including interference or derivation proceedings before the USPTO, or oppositions and other proceedings in foreign jurisdictions. We may be exposed to, or threatened with, future litigation by third parties having patent or other intellectual property rights alleging that our product candidates, manufacturing methods, formulations, administration methods and/or proprietary technologies infringe, misappropriate or otherwise violate their intellectual property rights.

Numerous issued patents and pending patent applications that are owned by third parties exist in the fields in which we are developing our product candidates. As the biotechnology and pharmaceutical industries expand and more patents are issued, the risk increases that our product candidates may give rise to claims of infringement of the patent rights of others. Moreover, it is not always clear to industry participants, including us, the claim scope that may issue from pending patent applications owned by third parties or which patents cover various types of drugs, products or their methods of use or manufacture. Thus, because of the large number of patents issued and patent applications filed in our fields, there may be a risk that third parties, including our competitors, may allege they have patent rights encompassing our product candidates, technologies or methods and that we are employing their proprietary technology without authorization.

If we were sued for patent infringement, we would need to demonstrate that the relevant product or methods of using the product either do not infringe the patent claims of the relevant patent or that the patent claims are invalid or unenforceable, and we may not be able to do this. In order to successfully challenge the validity of any such United States patent in federal court, we would need to overcome a presumption of validity. As this burden is high and requires us to present clear and convincing evidence as to the invalidity of any such United States patent claim, there is no assurance that a court of competent jurisdiction would agree with us and invalidate the claims of any such United States patent. Moreover, given the vast number of patents in our field of technology, we cannot be certain that we do not infringe existing patents or that we will not infringe patents that may be granted in the future.

While we may decide to initiate proceedings to challenge the validity of these or other patents in the future, we may be unsuccessful, and courts or patent offices in the United States and abroad could uphold the validity of any such patent. Furthermore, because patent applications can take many years to issue and may be confidential for 18 months or more after filing, and because pending patent claims can be revised before issuance, there may be applications now pending which may later result in issued patents that may be infringed by the manufacture, use or sale of our product candidates. Regardless of when filed, we may fail to identify relevant third-party patents or patent applications, or we may incorrectly conclude that a third-party patent is invalid or not infringed by our product candidates or activities. If a patent holder believes that one of our product candidates infringes its

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patent, the patent holder may sue us even if we have received patent protection for our technology. Moreover, we may face patent infringement claims from non-practicing entities that have no relevant drug revenue and against whom our own patent portfolio may thus have no deterrent effect. If a patent infringement suit were threatened or brought against us, we could be forced to stop or delay research, development, manufacturing or sales of the drug or product candidate that is the subject of the actual or threatened suit.

If any third-party patents are held by a court of competent jurisdiction to be valid and enforceable and to cover any of our technology or product candidates, including the manufacturing process of our product candidates, constructs or molecules used in or formed during the manufacturing process, or any final product itself, we could be forced, including by court order, to cease developing, manufacturing or commercializing the infringing product. Alternatively, we may be required to obtain a license from such third party in order to use the infringing technology and continue developing, manufacturing or marketing the infringing product. If we were required to obtain a license to continue to manufacture or market the affected product, we may be required to pay substantial royalties or grant cross-licenses to our patents. We cannot, however, assure that any such license will be available on acceptable terms, if at all. Ultimately, we could be prevented from commercializing a product, or be forced to cease some aspect of our business operations as a result of claims of patent infringement or violation of other intellectual property rights. Further, the outcome of intellectual property litigation is subject to uncertainties that cannot be adequately quantified in advance, including the demeanor and credibility of witnesses and the identity of any adverse party. This is especially true in intellectual property cases that may turn on the testimony of experts as to technical facts upon which experts may reasonably disagree. Furthermore, we may not be able to obtain any required license on commercially reasonable terms or at all. Even if we were able to obtain a license, it could be non-exclusive, thereby giving our competitors access to the same technologies licensed to us; alternatively, or additionally, it could include terms that impede or destroy our ability to compete successfully in the commercial marketplace. In addition, we could be found liable for significant monetary damages, including treble damages and attorneys' fees if we are found to have willfully infringed a patent. A finding of infringement could prevent us from commercializing a product or force us to cease some of our business operations, which could harm our business. Claims that we have misappropriated the confidential information or trade secrets of third parties could have a similar negative impact on our business. Furthermore, because of the substantial amount of discovery required in connection with intellectual property litigation or administrative proceedings, there is a risk that some of our confidential information could be compromised by disclosure. In addition, any uncertainties resulting from the initiation and continuation of any litigation could have material adverse effect on our ability to raise additional funds or otherwise have a material adverse effect on our business, results of operations, financial condition and prospects.

The cost to us in defending or initiating any litigation or other proceeding relating to patent or other proprietary rights, even if resolved in our favor, could be substantial, and litigation would divert our management's attention. Some of our competitors may be able to sustain the costs of complex patent litigation more effectively than we can because they have substantially greater resources. Uncertainties resulting from the initiation and continuation of patent litigation or other proceedings could delay our research and development efforts and limit our ability to continue our operations.

We are currently, and may in the future be, subject to claims that we and our employees, consultants, or independent contractors have wrongfully used or disclosed confidential information or trade secrets of third parties.

We employ individuals who were previously employed at other biotechnology or biopharmaceutical companies. Although we try to ensure that our employees, consultants and advisors do not use the proprietary information or know-how of others in their work for us, we may be subject to claims that we or our employees, consultants, or independent contractors have inadvertently or otherwise used or disclosed confidential information of our employees' former employers or other third parties. We may also be subject to claims that former employers or other third parties have an ownership interest in our future patents. Litigation may be necessary to defend against these claims. There is no guarantee of success in defending these claims, and even if

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we are successful, litigation can have an adverse impact on us due to defense and settlement costs, diversion of management resources, harm to our reputation and other factors. For example, on October 12, 2023, Rocket filed a lawsuit against us and two individuals claiming, among other things, misappropriation of confidential information and trade secrets. The individual defendants are a current employee and a former employee of our analytical development team, both of whom were employed at Rocket before joining us in 2021. The complaint alleges the individual defendants downloaded confidential Rocket company documents and other proprietary materials prior to leaving Rocket in 2021 and that we used this information to advance our programs. The complaint seeks unspecified damages and asks the court to enjoin us from competing and working in the market for gene therapy treatments targeting cardiac diseases. We retained legal counsel to assist with our ongoing review of the allegations in Rocket's complaint and are confident in our defenses to the allegations. On December 7, 2023, we filed a motion to dismiss the complaint, and the motion is fully briefed and pending before the court. It is not possible to predict the outcome with certainty and an estimate of the possible loss cannot be made. For additional information regarding this litigation, see the section titled "*Legal Proceedings*".

Even if we are successful in defending against these types of claims, litigation or other legal proceedings relating to intellectual property claims may cause us to incur significant expenses and could distract our technical and management personnel from their normal responsibilities. In addition, there could be public announcements of the results of hearings, motions or other interim proceedings or developments, and, if securities analysts or investors perceive these results to be negative, that perception could have a substantial adverse effect on the price of our Common Stock. This type of litigation or proceeding could substantially increase our operating losses and reduce our resources available for development activities. Some of our competitors may be able to sustain the costs of this type of litigation or proceedings more effectively than we can because of their substantially greater financial resources. Uncertainties resulting from the initiation and continuation of intellectual property litigation or other intellectual property related proceedings could adversely affect our ability to compete in the marketplace.

We may be subject to claims challenging the inventorship or ownership of our future patents and other intellectual property.

We may also be subject to claims that former employees, collaborators, or other third parties have an ownership interest in our patent applications, our future patents, or other intellectual property. We may be subject to ownership disputes in the future arising, for example, from conflicting obligations of consultants or others who are involved in developing our product candidates and platform discovery. Although it is our policy to require our employees and contractors who may be involved in the conception or development of intellectual property to execute agreements assigning such intellectual property to us, we may be unsuccessful in executing such an agreement with each party who, in fact, conceives or develops intellectual property that we regard as our own, and we cannot be certain that our agreements with such parties will be upheld in the face of a potential challenge, or that they will not be breached, for which we may not have an adequate remedy. The assignment of intellectual property rights may not be self-executing, or the assignment agreements may be breached, and litigation may be necessary to defend against these and other claims challenging inventorship or ownership. If we fail in defending any such claims, in addition to paying monetary damages, we may lose valuable intellectual property rights, such as exclusive ownership of, or right to use, valuable intellectual property. Such an outcome could have a material adverse effect on our business. Even if we are successful in defending against such claims, litigation could result in substantial costs and be a distraction to management and other employees.

Reliance on third parties requires us to share our trade secrets, which increases the possibility that a competitor will discover them or that our trade secrets will be misappropriated or disclosed.

If we rely on third parties to manufacture or commercialize our product candidates, or if we collaborate with additional third parties for the development of such product candidates, we must, at times, share trade secrets with them. We may also conduct joint research and development programs that may require us to share trade secrets under the terms of our research and development partnerships or similar agreements. We seek to protect

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our proprietary technology in part by entering into confidentiality agreements and, if applicable, material transfer agreements, consulting agreements or other similar agreements with our advisors, employees, third-party contractors and consultants prior to beginning research or disclosing proprietary information. These agreements typically limit the rights of the third parties to use or disclose our confidential information, including our trade secrets. Despite the contractual provisions employed when working with third parties, the need to share trade secrets and other confidential information increases the risk that such trade secrets become known by our competitors, are inadvertently incorporated into the technology of others, or are disclosed or used in violation of these agreements. Given that our proprietary position is based, in part, on our know-how and trade secrets, a competitor's discovery of our trade secrets or other unauthorized use or disclosure could have an adverse effect on our business and results of operations.

In addition, these agreements typically restrict the ability of our advisors, employees, third-party contractors and consultants to publish data potentially relating to our trade secrets. Despite our efforts to protect our trade secrets, we may not be able to prevent the unauthorized disclosure or use of our technical know-how or other trade secrets by the parties to these agreements. Moreover, we cannot guarantee that we have entered into such agreements with each party that may have or have had access to our confidential information or proprietary technology and processes. Monitoring unauthorized uses and disclosures is difficult, and we do not know whether the steps we have taken to protect our proprietary technologies will be effective. If any of the collaborators, scientific advisors, employees, contractors and consultants who are parties to these agreements breaches or violates the terms of any of these agreements, we may not have adequate remedies for any such breach or violation, and we could lose our trade secrets as a result. Moreover, if confidential information that is licensed or disclosed to us by our partners, collaborators, or others is inadvertently disclosed or subject to a breach or violation, we may be exposed to liability to the owner of that confidential information. Enforcing a claim that a third party illegally obtained and is using our trade secrets, like patent litigation, is expensive and time consuming, and the outcome is unpredictable. In addition, courts outside the United States are sometimes less willing to protect trade secrets.

Intellectual property rights do not necessarily address all potential threats to our competitive advantage.

The degree of future protection afforded by our intellectual property rights is uncertain because intellectual property rights have limitations, and may not adequately protect our business, or permit us to maintain our competitive advantage. The following examples are illustrative:

- others may be able to make or use capsids, nucleic acids and vectors that are similar to the biological compositions of our products that are the same as or similar to our product candidates but that are not covered by the claims of owned or in-licensed patents;
- others may independently develop similar or alternative technologies or duplicate any of our technologies;
- it is possible that others may circumvent our owned or in-licensed patents;
- others, including inventors or developers of our owned or in-licensed patented technologies who may become involved with competitors, may independently develop similar technologies that function as alternatives or replacements for any of our technologies without infringing our intellectual property rights;
- it is possible that our owned or in-licensed patents or patent applications omit individual(s) who should be listed as inventor(s) or include individual(s) who should not be listed as inventor(s), which may cause these patents or patents issuing from these patent applications to be held invalid or unenforceable;
- we or our licensors or our other collaboration partners might not have been the first to conceive and reduce to practice the inventions covered by the patents or patent applications that we own, license or will own or license;

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- we or our licensors or our other collaboration partners might not have been the first to file patent applications covering certain of the patents or patent applications that we or they own or have obtained a license, or will own or will have obtained a license;
- we or our licensors may fail to meet obligations to the U.S. government with respect to in-licensed patents and patent applications funded by U.S. government grants, leading to the loss of patent rights;
- it is possible that our pending patent applications will not result in issued patents;
- we may not be able to generate sufficient data to support full patent applications that protect the entire breadth of developments in one or more of our programs;
- no patent protection may be available with regard to formulation or method of use;
- the claims of our owned or in-licensed issued patents or patent applications, if and when issued, may not cover our product candidates;
- it is possible that there are prior public disclosures that could invalidate our or our licensors' patents;
- it is possible that there are unpublished applications or patent applications maintained in secrecy that may later issue with claims covering our products or technology similar to ours;
- issued patents that we own or exclusively license may not provide us with any competitive advantage, or may be held invalid or unenforceable, as a result of legal challenges by our competitors;
- we may not exclusively license our patents and, therefore, may not have a competitive advantage if such patents are licensed to others;
- our competitors might conduct research and development activities in countries where we do not have patent rights, or in countries where research and development safe harbor laws exist, and then use the information learned from such activities to develop competitive products for sale in our major commercial markets;
- the laws of other countries may not protect our or our licensors', as the case may be, proprietary rights to the same extent as the laws of the United States;
- there may be significant pressure on the U.S. government and international governmental bodies to limit the scope of patent protection both inside and outside the United States for disease treatments that prove successful, as a matter of public policy regarding worldwide health concerns;
- countries other than the United States may, under certain circumstances, force us or our licensors to grant a license under our patents to a competitor, thus allowing the competitor to compete with us in that jurisdiction or forcing us to lower the price of our drug in that jurisdiction;
- we have engaged in scientific collaborations in the past and will continue to do so in the future and our collaborators may develop adjacent or competing products that are outside the scope of our patents;
- we may not successfully commercialize the product candidates, if approved, before our relevant patents expire;
- we may not develop additional proprietary technologies for which we can obtain patent protection;
- it is possible that product candidates or technologies we develop may be covered by third parties' patents or other exclusive rights;
- ownership, validity or enforceability of our or our licensors' patents or patent applications may be challenged by third parties; and
- the patents of third parties or pending or future applications of third parties, if issued, may have an adverse effect on our business.

We may have limited geographical protection with respect to certain patents and we may not be able to protect our intellectual property rights throughout the world.

Filing and prosecuting patent applications and defending patents covering our product candidates in all countries throughout the world would be prohibitively expensive. Competitors may use our technologies in jurisdictions where we have not obtained patent protection to develop their own products and, further, may export otherwise infringing products to territories where we have patent protection, but enforcement rights are not as strong as that in the United States or Europe. These products may compete with our product candidates, and our future patents or other intellectual property rights may not be effective or sufficient to prevent them from competing.

In addition, we may decide to abandon national and regional patent applications before they are granted. The examination of each national or regional patent application is an independent proceeding. As a result, patent applications in the same family may issue as patents in some jurisdictions, such as in the United States, but may issue as patents with claims of different scope or may even be refused in other jurisdictions. It is also quite common that depending on the country, the scope of patent protection may vary for the same product candidate or technology.

While we intend to protect our intellectual property rights in our expected significant markets, we cannot ensure that we will be able to initiate or maintain similar efforts in all jurisdictions in which we may wish to market our product candidates. Accordingly, our efforts to protect our intellectual property rights in such countries may be inadequate, which may have an adverse effect on our ability to successfully commercialize our product candidates in all of our expected significant foreign markets. If we encounter difficulties in protecting, or are otherwise precluded from effectively protecting, the intellectual property rights important for our business in such jurisdictions, the value of these rights may be diminished, and we may face additional competition from others in those jurisdictions.

The laws of some jurisdictions do not protect intellectual property rights to the same extent as the laws or rules and regulations in the United States and Europe and many companies have encountered significant difficulties in protecting and defending such rights in such jurisdictions. The legal systems of certain countries, particularly certain developing countries, do not favor the enforcement of patents, trade secrets and other intellectual property rights, which could make it difficult for us to stop the infringement of our future patents or marketing of competing products in violation of our proprietary rights generally. Proceedings to enforce our patent rights in other jurisdictions, whether or not successful, could result in substantial costs and divert our efforts and attention from other aspects of our business, could put our future patents at risk of being invalidated or interpreted narrowly and our patent applications at risk of not issuing as patents, and could provoke third parties to assert claims against us. We may not prevail in any lawsuits that we initiate, and the damages or other remedies awarded, if any, may not be commercially meaningful. Accordingly, our efforts to enforce our intellectual property rights around the world may be inadequate to obtain a significant commercial advantage from the intellectual property that we develop or license.

Some countries also have compulsory licensing laws under which a patent owner may be compelled to grant licenses to third parties. In addition, some countries limit the enforceability of patents against government agencies or government contractors. In those countries, the patent owner may have limited remedies, which could materially diminish the value of such patents. If we are forced to grant a license to third parties with respect to any patents relevant to our business, our competitive position may be impaired.

Obtaining and maintaining our patent protection depends on compliance with various procedural, document submission, fee payment and other requirements imposed by government patent agencies, and our patent protection could be reduced or eliminated for non-compliance with these requirements.

Periodic maintenance fees, renewal fees, annuity fees and various other government fees on patents and/or applications will be due to be paid to the USPTO and various government patent agencies outside of the United

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States over the lifetime of our patents and/or applications and any patent rights we may obtain in the future. Furthermore, the USPTO and various non-United States government patent agencies require compliance with several procedural, documentary, fee payment and other similar provisions during the patent application process. In many cases, an inadvertent lapse of a patent or patent application can be cured by payment of a late fee or by other means in accordance with the applicable rules. There are situations, however, in which non-compliance can result in abandonment or lapse of the patents or patent applications, resulting in partial or complete loss of patent rights in the relevant jurisdiction. In such an event, potential competitors might be able to enter the market, which could have a material adverse effect on our business.

Changes in patent laws or patent jurisprudence could diminish the value of patents in general, thereby impairing our ability to protect our product candidates.

As is the case with other biotechnology companies, our success is heavily dependent on intellectual property, particularly patents. Obtaining and enforcing patents in the biotechnology and genetic medicine industries involve both technological and legal complexity. Therefore, obtaining and enforcing biotechnology and genetic medicine patents is costly, time-consuming and inherently uncertain. In addition, the Leahy-Smith America Invents Act (AIA), which was passed in September 2011, resulted in significant changes to the U.S. patent system.

An important change introduced by the AIA is that, as of March 16, 2013, the United States transitioned from a “first-to-invent” to a “first-to-file” system for deciding which party should be granted a patent when two or more patent applications are filed by different parties claiming the same invention. Under a “first-to-file” system, assuming the other requirements for patentability are met, the first inventor to file a patent application generally will be entitled to a patent on the invention regardless of whether another inventor had made the invention earlier. A third party that files a patent application in the USPTO after that date but before us could therefore be awarded a patent covering an invention of ours even if we made the invention before it was made by the third party. This will require us to be cognizant going forward of the time from invention to filing of a patent application and be diligent in filing patent applications, but circumstances could prevent us from promptly filing patent applications on our inventions.

Among some of the other changes introduced by the AIA are changes that limit where a patentee may file a patent infringement suit and providing opportunities for third parties to challenge any issued patent in the USPTO. This applies to all of our U.S. patents, even those issued before March 16, 2013. Because of a lower evidentiary standard in USPTO proceedings compared to the evidentiary standard in U.S. federal courts necessary to invalidate a patent claim, a third party could potentially provide evidence in a USPTO proceeding sufficient for the USPTO to hold a claim invalid even though the same evidence would be insufficient to invalidate the claim if first presented in a district court action.

Accordingly, a third party may attempt to use the USPTO procedures to invalidate our patent claims that would not have been invalidated if first challenged by the third party as a defendant in a district court action. It is not clear what, if any, impact the AIA will have on the operation of our business. However, the AIA and its implementation could increase the uncertainties and costs surrounding the prosecution of our or our licensors’ patent applications and the enforcement or defense of our or our licensors’ issued patents.

We may become involved in opposition, interference, derivation, inter partes review or other proceedings challenging our or our licensors’ patent rights, and the outcome of any proceedings are highly uncertain. An adverse determination in any such proceeding could reduce the scope of, or invalidate, our owned or in-licensed patent rights, allow third parties to commercialize our technology or products and compete directly with us, without payment to us, or result in our inability to manufacture or commercialize products without infringing third-party patent rights.

In addition, the United States federal government retains certain rights in inventions produced with its financial assistance under the Bayh-Dole Act. The federal government retains a “nonexclusive, nontransferable,

irrevocable, paid-up license” for its own benefit. The Bayh-Dole Act also provides federal agencies with “march-in rights.” March-in rights allows the government, in specified circumstances, to require the contractor or successors in title to the patent to grant a “nonexclusive, partially exclusive, or exclusive license” to a “responsible applicant or applicants.” If the patent owner refuses to do so, the government may grant the license itself. Some of our licensed patents are subject to the provisions of the Bayh-Dole Act. If our licensors fail to comply with the regulations of the Bayh-Dole Act, they could lose title to any patents subject to such regulations, which could affect our license rights under the patents and our ability to stop others from using or commercializing similar or identical technology and products, or limit patent protection for our technology and products.

Additionally, the U.S. Supreme Court has ruled on several patent cases in recent years either narrowing the scope of patent protection available in certain circumstances or weakening the rights of patent owners in certain situations, and there are other open questions under patent law that courts have yet to decisively address. In addition to increasing uncertainty with regard to our ability to obtain patents in the future, this combination of events has created uncertainty with respect to the value of patents, once obtained. Depending on decisions by Congress, the federal courts and the USPTO, the laws and regulations governing patents could change in unpredictable ways and could weaken our ability to obtain new patents or to enforce our existing patents and patents that we might obtain in the future. In addition, the European patent system is relatively stringent in the type of amendments that are allowed during prosecution, but the complexity and uncertainty of European patent laws has also increased in recent years. Complying with these laws and regulations could limit our ability to obtain new patents in the future that may be important for our business.

If our trademarks and trade names are not adequately protected, then we may not be able to build name recognition in our markets of interest and our business may be adversely affected.

Our current or future trademarks or trade names may be challenged, infringed, circumvented or declared generic or descriptive or determined to be infringing on other marks. We may not be able to protect our rights to these trademarks and trade names or may be forced to stop using these names, which we need for name recognition by potential partners or customers in our markets of interest. During trademark registration proceedings, we may receive rejections of our applications by the USPTO or in other foreign jurisdictions. Although we would be given an opportunity to respond to those rejections, we may be unable to overcome such rejections. In addition, in the USPTO and in comparable agencies in many foreign jurisdictions, third parties are given an opportunity to oppose pending trademark applications and to seek to cancel registered trademarks. Opposition or cancellation proceedings may be filed against our trademarks, and our trademarks may not survive such proceedings. If we are unable to establish name recognition based on our trademarks and trade names, we may not be able to compete effectively and our business may be adversely affected. We may license our trademarks and trade names to third parties, such as distributors. Although these license agreements may provide guidelines for how our trademarks and trade names may be used, a breach of these agreements or misuse of our trademarks and tradenames by our licensees may jeopardize our rights in or diminish the goodwill associated with our trademarks and trade names.

Moreover, any name we have proposed to use with our product candidate in the United States must be approved by the FDA, regardless of whether we have registered it, or applied to register it, as a trademark. Similar requirements exist in Europe. The FDA typically conducts a review of proposed product names, including an evaluation of potential for confusion with other product names. If the FDA (or an equivalent administrative body in a foreign jurisdiction) objects to any of our proposed proprietary product names, we may be required to expend significant additional resources in an effort to identify a suitable substitute name that would qualify under applicable trademark laws, not infringe the existing rights of third parties and be acceptable to the FDA. Furthermore, in many countries, owning and maintaining a trademark registration may not provide an adequate defense against a subsequent infringement claim asserted by the owner of a senior trademark. At times, competitors or other third parties may adopt trade names or trademarks similar to ours, thereby impeding our ability to build brand identity and possibly leading to market confusion. In addition, there could be potential

trade name or trademark infringement claims brought by owners of other registered trademarks or trademarks that incorporate variations of our registered or unregistered trademarks or trade names. If we assert trademark infringement claims, a court may determine that the marks we have asserted are invalid or unenforceable, or that the party against whom we have asserted trademark infringement has superior rights to the marks in question. In this case, we could ultimately be forced to cease use of such trademarks.

Risks related to legal and regulatory compliance matters

Our current and future relationships with customers, healthcare providers, including physicians, and third-party payors may be subject, directly or indirectly, to federal and state healthcare fraud and abuse laws, false claims laws, health information privacy and security laws and other healthcare laws and regulations. If we are unable to comply, or have not fully complied, with such laws, we could face substantial penalties.

We are currently or will in the future be subject to healthcare regulation and enforcement by the U.S. federal government and the states in which we will conduct our business once our product candidates are approved by the FDA and commercialized in the United States. In addition to the FDA's restrictions on marketing of pharmaceutical products, the U.S. healthcare laws and regulations that may affect our ability to operate include: the federal fraud and abuse laws, including the federal anti-kickback and false claims laws; federal data privacy and security laws; and federal transparency laws related to payments and/or other transfers of value made to physicians and other healthcare professionals and teaching hospitals. For more information, see the section titled "*Business — Government Regulation — Other Healthcare Laws and Compliance Requirements.*" Many states have similar laws and regulations that may differ from each other and federal law in significant ways, thus complicating compliance efforts. For example, states have anti-kickback and false claims laws that may be broader in scope than analogous federal laws and may apply regardless of payor. In addition, state data privacy laws that protect the security of health information may differ from each other and may not be preempted by federal law. Moreover, several states have enacted legislation requiring pharmaceutical manufacturers to, among other things, establish marketing compliance programs, file periodic reports with the state, make periodic public disclosures on sales and marketing activities, report information related to drug pricing, require the registration of sales representatives, and prohibit certain other sales and marketing practices. These laws may adversely affect our sales, marketing and other activities with respect to any product candidate for which we receive approval to market in the United States by imposing administrative and compliance burdens on us. It is possible that governmental authorities will conclude that our current or future business practices may not comply with current or future statutes, regulations or case law involving applicable fraud and abuse or other healthcare laws and regulations. If our operations are found to be in violation of any of these laws or any other governmental regulations that may apply to us, we may be subject to significant penalties, including, without limitation, civil, criminal and administrative penalties, damages, fines, disgorgement, individual imprisonment, exclusion from participating in federal and state funded healthcare programs, such as Medicare and Medicaid, additional reporting requirements and oversight if we become subject to a corporate integrity agreement or similar agreement to resolve allegations of non-compliance with these laws, contractual damages, diminished profits and future earnings, reputational harm and the curtailment or restructuring of our operations, any of which could harm our business.

The risk of being found in violation of these laws is increased by the fact that many of them have not been fully interpreted by the regulatory authorities or the courts, and their provisions are open to a variety of interpretations. Efforts to ensure that our business arrangements with third parties will comply with applicable healthcare laws and regulations will involve substantial costs. Any action against us for violation of these laws, even if we successfully defend against it, could cause us to incur significant legal expenses and divert our management's attention from the operation of our business. The shifting compliance environment and the need to build and maintain robust and expandable systems to comply with multiple jurisdictions with different compliance and/or reporting requirements increases the possibility that a healthcare company may run afoul of one or more of the requirements.

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Even if we obtain FDA or EMA approval for any of our product candidates in the United States or European Union, we may never obtain approval for or commercialize any of them in any other jurisdiction, which would limit our ability to realize their full market potential.

In order to market any products in any particular jurisdiction, we must establish and comply with numerous and varying regulatory requirements on a country-by-country basis regarding safety and efficacy.

Approval by the FDA in the United States or the EMA in the European Union does not ensure approval by regulatory authorities in other countries or jurisdictions. However, the failure to obtain approval in one jurisdiction may negatively impact our ability to obtain approval elsewhere. In addition, clinical trials conducted in one country may not be accepted by regulatory authorities in other countries, and regulatory approval in one country does not guarantee regulatory approval in any other country.

Approval processes vary among countries and can involve additional product testing and validation and additional administrative review periods. Seeking foreign regulatory approval could result in difficulties and increased costs for us and require additional preclinical studies or clinical trials which could be costly and time consuming. Regulatory requirements can vary widely from country to country and could delay or prevent the introduction of our products in those countries. We do not have any product candidates approved for sale in any jurisdiction, including in international markets. If we fail to comply with regulatory requirements in international markets or to obtain and maintain required approvals, or if regulatory approvals in international markets are delayed, our target market will be reduced and our ability to realize the full market potential of any product we develop will be unrealized.

Even if we receive regulatory approval of our product candidates, we will be subject to ongoing regulatory obligations and continued regulatory review, which may result in significant additional expense, and we may be subject to penalties if we fail to comply with regulatory requirements or experience unanticipated problems with our product candidates.

Any product candidate for which we obtain marketing approval will be subject to ongoing regulatory requirements for, among other things, manufacturing processes, submission of post-approval clinical data and safety information, labeling, packaging, distribution, adverse event reporting, storage, recordkeeping, export, import, advertising, promotional activities and product tracking and tracing. These requirements include submissions of safety and other post-marketing information and reports, establishment registration and drug listing requirements, applicable tracking and tracing requirements, continued compliance with cGMP requirements relating to manufacturing, quality control, quality assurance and corresponding maintenance of records and documents, requirements regarding the distribution of samples to physicians and recordkeeping and cGCP requirements for any clinical trials that we conduct post-approval.

Any regulatory approvals that we receive for our product candidates or any future product candidates may also be subject to a REMS, limitations on the approved indicated uses for which the product may be marketed or to the conditions of approval, or requirements that we conduct potentially costly post-marketing testing, including Phase 4 trials and surveillance to monitor the quality, safety and efficacy of the product. An unsuccessful post-marketing study or failure to complete such a study could result in the withdrawal of marketing approval. We will further be required to immediately report any serious and unexpected adverse events and certain quality or production problems with our products to regulatory authorities along with other periodic reports.

The FDA and EMA closely regulate the post-approval marketing and promotion of genetic therapy medicines to ensure they are marketed only for the approved indications and in accordance with the provisions of the approved labeling. However, companies may share truthful and not misleading information that is otherwise consistent with a product's FDA approved labeling. The FDA imposes stringent restrictions on manufacturers' communications regarding off-label use and if we market our products for uses beyond their approved diseases,

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we may be subject to enforcement action for off-label marketing. Violations of the FDCA, relating to the promotion of prescription drugs for unapproved uses may lead to enforcement actions and investigations alleging violations of federal and state health care fraud and abuse laws, as well as state consumer protection laws. The holder of an approved BLA must submit new or supplemental applications and obtain prior approval for certain changes to the approved product, product labeling, or manufacturing process. A company that is found to have improperly promoted off-label uses of their products may be subject to significant civil, criminal and administrative penalties.

In addition, later discovery of previously unknown adverse events or other problems with our products, manufacturers or manufacturing processes, including adverse events of unanticipated severity or frequency, or with our third-party manufacturers or manufacturing processes, or failure to comply with regulatory requirements, may yield various results, including:

- restrictions on manufacturing such products;
- restrictions on the labeling or marketing of a product;
- restrictions on product distribution or use;
- refusal to allow entry into supply contracts, including government contracts;
- requirements to conduct post-marketing studies or clinical trials;
- warning or untitled letters, or holds on clinical trials;
- withdrawal of the products from the market;
- refusal to approve pending applications or supplements to approved applications that we submit;
- recall of products;
- fines, restitution or disgorgement of profits or revenues;
- suspension or withdrawal of marketing approvals;
- refusal to permit the import or export of our products;
- product seizure or detention; or
- injunctions or the imposition of administrative, civil or criminal penalties or monetary fines.

The FDA's policies, and the policies of foreign regulatory agencies, may change and additional government regulations may be enacted that could prevent, limit or delay regulatory approval of our product candidates.

We also cannot predict the likelihood, nature or extent of government regulation that may arise from future legislation or administrative or executive action, either in the United States or abroad. For example, executive orders or other actions could impose significant burdens on, or otherwise materially delay, FDA's ability to engage in routine oversight activities such as implementing statutes through rulemaking, issuance of guidance, and review and approval of marketing applications. If such executive actions were to impose restrictions on FDA's ability to engage in oversight and implementation activities in the normal course, our business could be negatively impacted. If we are slow or unable to adapt to changes in existing requirements or the adoption of new requirements or policies, or if we are not able to maintain regulatory compliance, we may lose any marketing approval that we may have obtained which would adversely affect our business, prospects and ability to achieve or sustain profitability.

Any government investigation of alleged violations of law could require us to expend significant time and resources in response and could generate negative publicity. The occurrence of any event or penalty described above may inhibit our ability to commercialize our current product candidates or any future product candidates and harm our business, financial condition, results of operations and prospects.

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Enacted and future healthcare legislation may increase the difficulty and cost for us to progress our clinical programs and obtain marketing approval of and commercialize our product candidates and may affect the prices we may set.

In the United States, the European Union and other jurisdictions, there have been, and we expect there will continue to be, a number of legislative and regulatory changes and proposed changes to the healthcare system that could affect our future results of operations. In particular, there have been and continue to be a number of initiatives at the U.S. federal and state levels that seek to reduce healthcare costs and improve the quality of healthcare. For more information, see the below section titled “*Business—Government Regulation—Healthcare Reform.*”

The continuing efforts of the government, insurance companies, managed care organizations and other payers of healthcare services to contain or reduce costs of healthcare may adversely affect:

- the demand for any of our product candidates, if approved;
- the ability to set a price that we believe is fair for any of our product candidates, if approved;
- our ability to generate revenues and achieve or maintain profitability;
- the level of taxes that we are required to pay; and
- the availability of capital.

Legislative and regulatory proposals have been made to expand post-approval requirements and restrict sales and promotional activities for pharmaceutical and biologic products. We cannot be sure whether additional legislative changes will be enacted, or whether FDA regulations, guidance or interpretations will be changed, or what the impact of such changes on the marketing approvals of our product candidates, if any, may be. In addition, increased scrutiny by Congress of the FDA’s approval process may significantly delay or prevent marketing approval, as well as subject us to more stringent product labeling and post-marketing testing and other requirements.

Moreover, payment methodologies may be subject to changes in healthcare legislation and regulatory initiatives. For example, CMS may develop new payment and delivery models, such as bundled payment models. In addition, recently there has been heightened governmental scrutiny over the manner in which manufacturers set prices for their marketed products, which has resulted in several U.S. presidential executive orders, Congressional inquiries and proposed and enacted federal legislation designed to, among other things, bring more transparency to drug pricing, reduce the cost of prescription drugs under Medicare, and review the relationship between pricing and manufacturer patient programs. We expect that additional U.S. federal healthcare reform measures will be adopted in the future, any of which could limit the amounts that the U.S. federal government will pay for healthcare products and services, which could result in reduced demand for our product candidates or additional pricing pressures and could negatively affect our customers and accordingly, our financial operations.

Individual states in the United States have also increasingly passed legislation and implemented regulations designed to control pharmaceutical and biological product pricing, including price or patient reimbursement constraints, discounts, restrictions on certain product access and marketing cost disclosure and transparency measures, and, in some cases, designed to encourage importation from other countries and bulk purchasing. Legally mandated price controls on payment amounts by third-party payors or other restrictions could harm our business, results of operations, financial condition and prospects. In addition, regional healthcare authorities and individual hospitals are increasingly using bidding procedures to determine what pharmaceutical products and which suppliers will be included in their prescription drug and other healthcare programs. This could reduce the ultimate demand for our product candidates or put pressure on our product pricing.

In the European Union, similar political, economic and regulatory developments may affect our ability to profitably commercialize our product candidates, if approved. In addition to continuing pressure on prices and

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cost containment measures, legislative developments at the European Union or member state level may result in significant additional requirements or obstacles that may increase our operating costs. The delivery of healthcare in the European Union, including the establishment and operation of health services and the pricing and reimbursement of medicines, is almost exclusively a matter for national, rather than European Union, law and policy. National governments and health service providers have different priorities and approaches to the delivery of health care and the pricing and reimbursement of products in that context. In general, however, the healthcare budgetary constraints in most EU member states have resulted in restrictions on the pricing and reimbursement of medicines by relevant health service providers. Coupled with ever-increasing European Union and national regulatory burdens on those wishing to develop and market products, this could prevent or delay marketing approval of our product candidates, restrict or regulate post-approval activities and affect our ability to commercialize our product candidates, if approved.

In markets outside of the United States and the European Union, reimbursement and healthcare payment systems vary significantly by country, and many countries have instituted price ceilings on specific products and therapies. We cannot predict the likelihood, nature or extent of government regulation that may arise from future legislation or administrative action in the United States, the European Union or any other jurisdiction. If we or any third parties we may engage are slow or unable to adapt to changes in existing requirements or the adoption of new requirements or policies, or if we or such third parties are not able to maintain regulatory compliance, our product candidates may lose any regulatory approval that may have been obtained and we may not achieve or sustain profitability.

Changes in funding for the FDA and other government agencies could hinder their ability to hire and retain key leadership and other personnel, or otherwise prevent new products and services from being developed or commercialized in a timely manner, which could negatively impact our business.

The ability of the FDA to review and approve new products can be affected by a variety of factors, including government budget and funding levels, ability to hire and retain key personnel, accept the payment of user fees, and statutory, regulatory and policy changes. Average review times at the agency have fluctuated in recent years as a result. In addition, government funding of other government agencies that fund research and development activities is subject to the political process, which is inherently fluid and unpredictable.

Disruptions at the FDA and other agencies may also slow the time necessary for new drugs to be reviewed and/or approved by necessary government agencies, which would adversely affect our business. For example, over the last several years, including for 35 days beginning on December 22, 2018, the U.S. government has shut down several times and certain regulatory agencies, such as the FDA, have had to furlough critical FDA employees and stop critical activities. Our business depends upon the ability of the FDA to accept and review our potential regulatory filings. If a prolonged government shutdown occurs, it could significantly impact the ability of the FDA to timely review and process our regulatory submissions, which could have a material adverse effect on our ability to advance clinical development of our product candidates.

If we are unable to establish sales, marketing and distribution capabilities either on our own or in collaboration with third parties, we may not be successful in commercializing our product candidates or realizing the synergies in the target diseases of our programs, even if they are approved.

We do not have any infrastructure for the sales, marketing or distribution of our products, and the cost of establishing and maintaining such an organization may exceed the cost-effectiveness of doing so. We expect to build a focused sales, distribution and marketing infrastructure to market our product candidates in the United States and European Union, if approved. There are significant expenses and risks involved with establishing our own sales, marketing and distribution capabilities, including our ability to hire, retain and appropriately incentivize qualified individuals, generate sufficient sales leads, provide adequate training to sales and marketing personnel, and effectively manage a geographically dispersed sales and marketing team. Any failure or delay in the development of our internal sales, marketing and distribution capabilities could delay any product launch,

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which would adversely impact the commercialization of our product candidates. Additionally, if the commercial launch of our product candidates for which we recruit a sales force and establish marketing capabilities is delayed or does not occur for any reason, we would have prematurely or unnecessarily incurred these commercialization expenses. This may be costly, and our investment would be lost if we cannot retain or reposition our sales and marketing personnel.

We may not have the resources in the foreseeable future to allocate to the sales and marketing of our product candidates in certain international markets. Therefore, our future sales in these markets will largely depend on our ability to enter into and maintain collaborative relationships for such capabilities, the collaborator's strategic interest in the product and such collaborator's ability to successfully market and sell the product. We may pursue collaborative arrangements regarding the sale and marketing of LX2006, LX1001 or LX2020, if approved, for certain markets overseas; however, we cannot assure that we will be able to establish or maintain such collaborative arrangements, or if able to do so, that they will have effective sales forces.

If we are unable to build our own sales force or negotiate a collaborative relationship for the commercialization of LX2006, LX1001 or LX2020, or any of our other product candidates, if approved, we may be forced to delay the potential commercialization of LX2006, LX1001 or LX2020 or any of our other product candidates or reduce the scope of our sales or marketing activities for LX2006, LX1001 or LX2020 or any of our other product candidates. If we elect to increase our expenditures to fund commercialization activities internationally, we will need to obtain additional capital, which may not be available to us on acceptable terms, or at all. We could enter into arrangements with collaborative partners at an earlier stage than otherwise would be ideal and we may be required to relinquish rights to LX2006, LX1001 or LX2020 or any of our other product candidates or otherwise agree to terms unfavorable to us, any of which may have an adverse effect on our business, operating results and prospects.

If we are unable to establish adequate sales, marketing and distribution capabilities, either on our own or in collaboration with third parties, we will not be successful in commercializing LX2006, LX1001 or LX2020 or any of our other product candidates, if approved, and may not become profitable and may incur significant additional losses. We will be competing with many companies that currently have extensive and well-funded marketing and sales operations. Without an internal team or the support of a third party to perform marketing and sales functions, we may be unable to compete successfully against these more established companies.

If we obtain approval to commercialize any products outside of the United States or the European Union, a variety of risks associated with international operations could adversely affect our business.

If LX2006, LX1001, LX2020 or any of our other product candidates are approved for commercialization, we may seek to enter into agreements with third parties to market them in certain jurisdictions outside the United States and the European Union. We expect that we would be subject to additional risks related to international pharmaceutical operations, including:

- different regulatory requirements for drug and biologic approvals and rules governing drug and biologic commercialization in foreign countries;
- reduced protection for intellectual property rights;
- foreign reimbursement, pricing and insurance regimes;
- unexpected changes in tariffs, trade barriers and regulatory requirements;
- economic weakness, including inflation, or political instability in particular foreign economies and markets;
- foreign currency fluctuations, which could result in increased operating expenses and reduced revenues, and other obligations incident to doing business in another country;

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- business interruptions resulting from geopolitical actions, including war and terrorism or natural disasters including earthquakes, typhoons, floods and fires, or from economic or political instability;
- greater difficulty with enforcing our contracts;
- potential noncompliance with the U.S. Foreign Corrupt Practices Act, or the FCPA, the U.K. Bribery Act 2010 and similar anti-bribery and anticorruption laws in other jurisdictions; and
- production shortages resulting from any events affecting raw material supply or manufacturing capabilities abroad.

In addition, there are complex regulatory, tax, labor and other legal requirements imposed by individual countries in Europe with which we will need to comply. If we are unable to successfully manage the challenges of international expansion and operations, our business and operating results could be harmed.

If we fail to comply with environmental, health and safety laws and regulations, we could become subject to fines or penalties or incur costs that could harm our business.

We are subject to numerous environmental, health and safety laws and regulations, including those governing laboratory procedures and the handling, use, storage, treatment and disposal of hazardous materials and wastes. From time to time and in the future, our operations may involve the use of hazardous and flammable materials, including chemicals and biological materials, and may also produce hazardous waste products. Even if we contract with third parties for the disposal of these materials and waste products, we cannot completely eliminate the risk of contamination or injury resulting from these materials. In the event of contamination or injury resulting from the use or disposal of our hazardous materials, we could be held liable for any resulting damages, and any liability could exceed our resources. We also could incur significant costs associated with civil or criminal fines and penalties for failure to comply with such laws and regulations.

We maintain workers' compensation insurance to cover us for costs and expenses we may incur due to injuries to our employees, but this insurance may not provide adequate coverage against potential liabilities. However, we do not maintain insurance for environmental liability or toxic tort claims that may be asserted against us.

In addition, we may incur substantial costs in order to comply with current or future environmental, health and safety laws and regulations. Environmental laws and regulations may impair our research, development or production efforts. In addition, failure to comply with these laws and regulations may result in substantial fines, penalties or other sanctions.

We are subject to a variety of privacy and data security laws, rules, regulations, policies, industry standards and contractual obligations, and our failure to comply with them could harm our business.

We maintain a large quantity of sensitive information, including confidential business and personal information in connection with the conduct of our clinical trials and related to our employees, and we are subject to laws and regulations governing the privacy and security of such information. In the United States, there are numerous federal and state privacy and data security laws and regulations governing the collection, use, disclosure and protection of personal information, including federal and state health information privacy laws, federal and state security breach notification laws and federal and state consumer protection laws. The legislative and regulatory landscape for privacy and data protection continues to evolve, and there has been an increasing focus on privacy and data protection issues, which may affect our business and is expected to increase our compliance costs and exposure to liability. In the United States, numerous federal and state laws and regulations could apply to our operations or the operations of our partners, including state and federal data breach notification laws, state health information privacy laws and federal and state consumer protection laws and regulations that govern the collection, use, disclosure and protection of health-related and other personal

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information. Among these regulations are: Section 5 of the Federal Trade Commission Act, which prohibits unfair or deceptive commercial practices; new rules adopted by the SEC in July 2023, which require public companies to disclose material cybersecurity incidents they experience and to disclose on an annual basis material information regarding their cybersecurity risk management, strategy, and governance; and HIPAA, as amended by HITECH, and the regulations promulgated thereunder. We may obtain health information from third parties, including research institutions from which we obtain clinical trial data, that are subject to privacy and security requirements under HIPAA, and depending on the facts and circumstances, we could be subject to significant penalties if we obtain, use or disclose individually identifiable health information in a manner that is not authorized or permitted by HIPAA.

In the European Economic Area, or EEA, and the United Kingdom, or UK, the collection, use, disclosure, transfer or other processing of personal data, including clinical trial data, of individuals is governed by the General Data Protection Regulation, or EU GDPR (with regards to the EEA) and UK GDPR (with regards to the UK), as well as applicable national data protection legislation and requirements. In this document, “GDPR” refers to both the EU GDPR and the UK GDPR, unless specified otherwise. The GDPR is wide ranging in scope imposes numerous requirements on companies that process personal data, including requirements relating to processing health and other sensitive data, obtaining consent of the individuals to whom the personal data relates, providing information to individuals regarding data processing activities, implementing safeguards to protect the security and confidentiality of personal data, providing notification of data breaches, and taking certain measures when engaging third-party processors. The GDPR imposes substantial fines for breaches and violations (up to the greater of €20 million (£17.5 million for the UK) or 4% of our consolidated annual worldwide gross revenue), and confers a private right of action on data subjects and consumer associations to lodge complaints with supervisory authorities, seek judicial remedies and obtain compensation for damages resulting from violations of the GDPR.

The GDPR also includes restrictions on cross-border data transfers of personal data to countries outside the EEA and the UK that are not considered by the European Commission and UK government as providing “adequate” protection to personal data, or third countries, including the United States, unless a valid GDPR transfer mechanism (for example, the European Commission approved Standard Contractual Clauses, or SCCs, and the UK International Data Transfer Agreement/Addendum, or UK IDTA) has been put in place. Where relying on the SCCs or UK IDTA for data transfers, we may also be required to carry out transfer impact assessments to assess whether the recipient is subject to local laws which allow public authority access to personal data. The international transfer obligations under the EEA and UK data protection regimes will require significant effort and cost, and may result in us needing to make strategic considerations around where EEA and UK personal data is transferred and which service providers we can utilize for the processing of EEA and UK personal data. Although the UK is regarded as a third country under the EU GDPR, the European Commission has issued a decision recognizing the UK as providing adequate protection under the EU GDPR, or Adequacy Decision, and, therefore, transfers of personal data originating in the EEA to the UK remain unrestricted.

The UK government has confirmed that personal data transfers from the UK to the EEA remain free flowing. The UK Government has also now introduced a Data Protection and Digital Information Bill, or UK Bill, into the UK legislative process. The aim of the UK Bill is to reform the UK’s data protection regime following Brexit. If passed, the final version of the UK Bill may have the effect of further altering the similarities between the UK and EEA data protection regime and threaten the UK Adequacy Decision from the European Commission. This may lead to additional compliance costs and could increase our overall risk. The respective provisions and enforcement of the EU GDPR and UK GDPR may further diverge in the future and create additional regulatory challenges and uncertainties.

Compliance with these and any other applicable privacy and data security laws and regulations is a rigorous and time-intensive process, and we may be required to put in place additional mechanisms ensuring compliance with the new data protection rules. If we fail to comply with any such laws or regulations, we may face significant fines and penalties that could adversely affect our business, financial condition and results of

operations. Furthermore, the laws are not consistent, and compliance in the event of a widespread data breach is costly. In addition, states are constantly adopting new laws or amending existing laws, requiring attention to frequently changing regulatory requirements. For example, California enacted the California Consumer Privacy Act, or CCPA, which took effect on January 1, 2020, became enforceable by the California Attorney General on July 1, 2020 and has been dubbed the first “GDPR-like” law in the United States. The CCPA gives California residents expanded rights to access and delete their personal information, opt out of certain personal information sharing and receive detailed information about how their personal information is used by requiring covered companies to provide new disclosures to California consumers (as that term is broadly defined) and provide such consumers new ways to opt out of certain sales of personal information. The CCPA provides for civil penalties for violations, as well as a private right of action for data breaches that is expected to increase data breach litigation. Further, the California Privacy Rights Act, or CPRA, which became effective as of January 1, 2023, amended the CCPA and imposes additional data protection obligations on companies doing business in California, including additional consumer rights processes, limitations on data uses, new audit requirements for higher risk data and opt outs for certain uses of sensitive data. The amendments introduced by the CPRA also created a new California data protection agency authorized to issue substantive regulations, and it is anticipated that this development could result in increased privacy and information security enforcement. Although the CCPA, as amended by the CPRA, currently exempts certain health-related information, including clinical trial data, the CCPA may increase our compliance costs and potential liability if we expand our operations into California. Similar broad consumer privacy laws have been enacted in Colorado, Connecticut, Virginia, Utah, Iowa and Indiana and have been proposed in numerous other states and at the federal level. If passed, these bills may have potentially conflicting requirements that would make compliance challenging.

In addition to these consumer privacy laws, the state of Washington recently enacted a comprehensive privacy bill, called the My Health My Data Act. Effective March 2024, this new law will impose strict requirements on the collection, use and processing of health related information that is not subject to HIPAA. Other states are considering bills with similar requirements. The Washington law and, if passed, the other state bills, will add additional complexity to our existing compliance obligations.

With the GDPR, CCPA and other laws, regulations and other obligations relating to privacy and data protection imposing new and relatively burdensome obligations, and with the substantial uncertainty over the interpretation and application of these and other obligations, we may face challenges in addressing their requirements and making necessary changes to our policies and practices and may incur significant costs and expenses in an effort to do so. We will continue to assess, develop, update, and adapt our practices, procedures, and policies in order to address existing and new requirements under applicable data privacy and protection laws and regulations. However, it is possible that both existing and new laws, regulations, and other obligations to which we are or may be subject, may be interpreted and applied in a manner that is inconsistent with our existing or future privacy and data protection practices. Any failure or perceived failure by us to comply with our obligations may result in governmental investigations or enforcement actions, litigation, claims, or public statements against us and could result in significant liability, cause harm to our brand and reputation, and otherwise materially and adversely affect our reputation and business. We do not currently have any formal data privacy policies and procedures in place and have not completed formal assessments of whether we are in compliance with all applicable data privacy laws and regulations. Additionally, if third parties with which we work, such as vendors or service providers, violate applicable laws, rules or regulations or our policies, such violations may also put our or our clinical trial and employee data, including personal data, at risk, which could in turn have an adverse effect on our business.

We are subject to U.S. and certain foreign anti-corruption laws and regulations, export and import controls, sanctions and embargoes. We could face liability and other serious consequences for violations.

We are subject to anti-corruption laws and regulations, including the FCPA, the U.S. domestic bribery statute contained in 18 U.S.C. § 201, the U.S. Travel Act and other state and national anti-bribery laws in the countries in which we may conduct activities in the future. Anti-corruption laws are interpreted broadly and

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generally prohibit companies and their employees, agents, contractors and other third-party collaborators from offering, promising, giving, or authorizing others to give anything of value, either directly or indirectly through third parties, to any person in the public or private sector to obtain or retain business. The FCPA also requires public companies to make and keep books and records that accurately and fairly reflect the transactions of the corporation and to devise and maintain an adequate system of internal accounting controls.

Our business is heavily regulated and therefore involves significant interaction with public officials, including officials of non-United States governments. Additionally, in many other countries, the healthcare providers who prescribe pharmaceuticals are employed by their government, and therefore will be considered foreign officials for purposes of the FCPA. We also expect to rely on third parties for research, preclinical studies and clinical trials and/or to obtain necessary permits, licenses, patent registrations and other marketing approvals. We can be held liable for the corrupt or other illegal activities of our employees, agents, CROs, contractors and other collaborators and partners, even if we do not explicitly authorize or have actual knowledge of such activities.

We are also subject to export control and import laws and regulations, including the U.S. Export Administration Regulations, U.S. Customs regulations and various economic and trade sanctions regulations administered by the U.S. Treasury Department's Office of Foreign Assets Controls. Compliance with applicable regulatory requirements regarding the export of our products may create delays in the introduction of our products in international markets or, in some cases, prevent the export of our products to some countries altogether. Furthermore, U.S. export control laws and economic sanctions prohibit the shipment of certain products and services to countries, governments and persons targeted by U.S. sanctions.

There is no certainty that all of our employees, agents, suppliers, manufacturers, contractors or collaborators, or those of our affiliates, will comply with all applicable anti-corruption, export and import control, and sanctions laws and regulations, particularly given the high level of complexity of these laws. Violations of these laws and regulations could result in fines, criminal sanctions against us, our officers, or our employees, the closing down of facilities, including those of our suppliers and manufacturers, requirements to obtain export licenses, cessation of business activities in sanctioned countries, implementation of compliance programs, and prohibitions on the conduct of our business. Any such violations could include prohibitions on our ability to offer our products in one or more countries as well as difficulties in manufacturing or continuing to develop our products, and could materially damage our reputation, our brand, our international expansion efforts, our ability to attract and retain employees, and our business, prospects, operating results and financial condition.

Risks related to employee matters and managing our growth

Our future success depends on our ability to attract and retain key executives and advisors and to attract, retain and motivate qualified personnel.

We are highly dependent on the management, development, clinical, financial and business development expertise of our executive officers, particularly R. Nolan Townsend, our Chief Executive Officer and a member of our board of directors, Eric Adler, M.D., our Chief Medical Officer and Head of Research, Sandi See Tai, M.D., our Chief Development Officer, as well as on the scientific expertise of our founder, Ronald G. Crystal, M.D., Professor and Chairman of Weill Cornell Medicine's Department of Genetic Medicine. Each of our executive officers may currently terminate their employment with us at any time and we do not have an employment contract with Dr. Crystal. We do not maintain "key person" insurance for any of our executives or employees.

Recruiting and retaining qualified executives, scientists and clinical personnel and, if we progress the development of our product pipeline toward scaling up for commercialization, manufacturing and sales and marketing personnel, will also be critical to our success. The loss of the services of our executive officers or other key employees, or our inability to recruit certain executives, could impede the achievement of our

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development and commercialization objectives and seriously harm our ability to successfully implement our business strategy. Furthermore, recruiting executive officers, or replacing executive officers and key employees may be difficult and may take an extended period of time because of the limited number of individuals in our industry with the breadth of skills and experience required to successfully develop, gain regulatory approval of and commercialize gene therapy products. Competition to hire from this limited pool is intense, and we have experienced and may continue to experience challenges filling certain executive roles. We may be unable to hire, train, retain or motivate key personnel on acceptable terms given the competition among numerous pharmaceutical and biotechnology companies for similar personnel. We also experience competition for the hiring of scientific and clinical personnel from universities and research institutions. In addition, we rely on consultants and advisors, including scientific and clinical advisors, to assist us in formulating our development and commercialization strategy. Our consultants and advisors may be employed by employers other than us and may have commitments under consulting or advisory contracts with other entities that may limit their availability to us. If we are unable to continue to attract and retain high quality personnel, our ability to pursue our growth strategy will be limited.

We expect to expand our clinical development, manufacturing and regulatory capabilities and potentially implement sales, marketing and distribution capabilities, and as a result, we may encounter difficulties in managing our growth, which could disrupt our operations.

As of December 31, 2023, we had 58 full-time employees. As our development progresses, we expect to experience significant growth in the number of our employees and the scope of our operations, particularly in the areas of clinical product development, regulatory affairs and, if any of our product candidates receives marketing approval, sales, marketing and distribution. To manage our anticipated future growth, we must continue to implement and improve our managerial, operational and financial systems, expand our facilities and continue to recruit and train additional qualified personnel. Due to our limited financial resources and the limited experience of our management team in managing a company with such anticipated growth, we may not be able to effectively manage the expansion of our operations or recruit and train additional qualified personnel. Our choice to focus on multiple therapeutic areas may negatively affect our ability to develop adequately the specialized capability and expertise necessary for operations. The expansion of our operations may lead to significant costs and may divert our management and business development resources. Any inability to manage growth could delay the execution of our business plans or disrupt our operations.

Our employees, independent contractors, consultants, collaborators, principal investigators, CROs, suppliers and vendors may be improperly classified and may engage in misconduct or other improper activities, including non-compliance with regulatory standards and requirements.

We endeavor to properly classify our employees as exempt or non-exempt with respect to wage and hour laws (including, but not limited to, for purposes of minimum wage, overtime and applicable meal and rest periods), and we monitor and evaluate such classifications. Although there are no current, pending, or threatened claims or investigations against us asserting that any employees have been incorrectly classified as exempt, the possibility nevertheless exists that certain job roles could be deemed to have been incorrectly classified as exempt. In addition, we endeavor to classify our workforce properly, and we monitor and evaluate such classifications. Although there are no current, pending, or threatened claims or investigations against us asserting that any independent contractors have been incorrectly classified, the possibility nevertheless exists that certain contractors could be deemed to be employees.

We are exposed to the risk that our employees, independent contractors, consultants, collaborators, principal investigators, CROs, suppliers and vendors may engage in fraudulent conduct or other illegal activity. Misconduct by these parties could include intentional, reckless and/or negligent conduct that violates FDA regulations, including those laws requiring the reporting of true, complete and accurate information to the FDA, manufacturing standards, federal and state healthcare laws and regulations, and laws that require the true, complete and accurate reporting of financial information or data. In particular, sales, marketing and business

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arrangements in the healthcare industry are subject to extensive laws and regulations intended to prevent fraud, kickbacks, self-dealing and other abusive practices. These laws and regulations may restrict or prohibit a wide range of pricing, discounting, marketing and promotion, sales commission, customer incentive programs and other business arrangements. Misconduct by these parties could also involve the improper use of individually identifiable information, including, without limitation, information obtained in the course of clinical trials, which could result in regulatory sanctions and serious harm to our reputation. Although we have adopted a code of business conduct and ethics, it is not always possible to identify and deter misconduct, and the precautions we take to detect and prevent this activity may not be effective in controlling unknown or unmanaged risks or losses or in protecting us from governmental investigations or other actions or lawsuits stemming from a failure to be in compliance with such laws or regulations. If any such actions are instituted against us, and we are not successful in defending ourselves or asserting our rights, those actions could have a significant impact on our business, including the imposition of significant civil, criminal and administrative penalties, including, without limitation, damages, fines, disgorgement, imprisonment, exclusion from participation in government healthcare programs, such as Medicare and Medicaid, additional reporting requirements and oversight if we become subject to a corporate integrity agreement or similar agreement to resolve allegations of non-compliance with these laws, and the curtailment or restructuring of our operations.

The administrator of our 2023 Equity Incentive Plan, or the 2023 Plan, is authorized to exercise its discretion to reprice stock options and stock appreciation rights, and if a repricing occurs, there may be adverse consequences to our business.

The administrator of the 2023 Plan, which is our compensation committee, is authorized, subject to the consent of any award holder whose award is materially impaired by such action, to reduce the exercise price of a stock option or stock appreciation right; to cancel a stock option or stock appreciation right in exchange for a different award, cash or other consideration; or to take any other action that is treated as a repricing under generally accepted accounting principles, or each such action, a repricing.

We have no current expectation that a repricing will occur. However, if the administrator were to implement a repricing without seeking prior stockholder approval, certain proxy advisory firms and/or institutional investors may express a lack of support for the repricing, and proxy advisory firms may recommend an “against” or “withhold” vote for members of our compensation committee or our board of directors. In addition, if we are required to hold an advisory vote on named executive officer compensation (known as a “say on pay” vote) at the time of, or subsequent to, any such repricing, it is likely, based on their current policies, that proxy advisory firms would issue an “against” recommendation on our say on pay proposal. Defending against negative recommendations with respect to our directors and/or say on pay proposal would require management attention, and could be costly and time-consuming.

If our stockholders agree with proxy advisory firms’ recommendations, we may need to make changes to our compensation and corporate governance practices, and perhaps the composition of our board of directors and its committees, potentially leading to business disruptions and a negative impact on our stock price. Even absent negative reactions from proxy advisory firms and institutional investors, we may be required to recognize a compensation expense and the repricing will require management’s time and attention and the payment of administrative costs and attorney and accounting firm fees. As such, a repricing could cause a negative impact on our stock price, and adverse consequences to our business.

Risks related to ownership of our Common Stock and our status as a public company

The trading price of the shares of our Common Stock may be volatile, and purchasers of our Common Stock could incur substantial losses.

The trading price of our Common Stock is likely to be highly volatile and could be subject to wide fluctuations in response to various factors, some of which are beyond our control, including limited trading

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volume. The stock market in general and the market for biopharmaceutical companies in particular have experienced extreme volatility that has often been unrelated to the operating performance of particular companies. As a result of this volatility, investors may not be able to sell their Common Stock at or above the price paid for the shares. The trading price for our Common Stock may be influenced by many factors, including those discussed in this “Risk Factors” section and elsewhere in this prospectus and:

- the reporting of unfavorable preclinical and clinical results;
- the commencement, enrollment or results of our clinical trials of LX2006, LX1001, LX2020 or any future clinical trials we may conduct, or changes in the development status of our product candidates;
- any delay in our regulatory filings for LX2006, LX1001, LX2020 or any other product candidate we may develop, and any adverse development or perceived adverse development with respect to the applicable regulatory authority’s review of such filings, including without limitation the FDA’s issuance of a “refusal to file” letter or a request for additional information;
- adverse results from, delays in or termination of clinical trials;
- adverse regulatory decisions, including failure to receive regulatory approval of our product candidates;
- unanticipated serious safety concerns related to the use of LX2006, LX1001 or LX2020 or any other product candidates;
- changes in financial estimates by us or by any equity research analysts who might cover our stock;
- conditions or trends in our industry;
- changes in the market valuations of similar companies;
- stock trading price and volume fluctuations of comparable companies and, in particular, those that operate in the biopharmaceutical industry;
- publication of research reports about us or our industry or positive or negative recommendations or withdrawal of research coverage by securities analysts;
- announcements by us or our competitors of significant acquisitions, strategic partnerships or divestitures;
- our relationships with our collaborators;
- announcements of investigations or regulatory scrutiny of our operations or lawsuits filed against us;
- investors’ general perception of our company and our business;
- recruitment or departure of key personnel;
- overall performance of the equity markets;
- trading volume of our Common Stock;
- disputes or other developments relating to proprietary rights, including patents, litigation matters and our ability to obtain patent protection for our technologies;
- significant lawsuits, including patent or stockholder litigation or employee or independent contractor litigation;
- changes in the structure of healthcare payment systems;
- unfavorable geopolitical and economic conditions; and
- other events or factors, many of which are beyond our control.

The global economy, including credit and financial markets and the banking sector, has experienced extreme volatility and disruptions, including, among other things, severely diminished liquidity and credit

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availability, declines in consumer confidence, declines in economic growth, increases in unemployment rates, supply chain shortages, increases in inflation rates, bank failures, higher interest rates and uncertainty about economic stability. For example, the ongoing wars in Ukraine and Israel have created volatility in the global capital markets and may have further global economic consequences, including disruptions of the global supply chain and energy markets. Any such volatility and disruptions may have adverse consequences on us or the third parties on whom we rely. If the equity and credit markets continue to deteriorate, it may make any necessary debt or equity financings more difficult to obtain in a timely manner or on favorable terms, more costly or more dilutive. Increased inflation rates can adversely affect us by increasing our costs, including labor and employee benefit costs. In addition, higher inflation and macro turmoil and uncertainty could also adversely affect our buyers and sellers, which could reduce demand for our products. These factors may negatively affect the trading price of our Common Stock, regardless of our actual operating performance.

In addition, in the past, stockholders have initiated class action lawsuits against pharmaceutical and biotechnology companies following periods of volatility in the trading prices of these companies' stock. This risk is especially relevant for us because biopharmaceutical companies have experienced significant stock price volatility in recent years. Such litigation, if instituted against us, could cause us to incur substantial costs and divert management's attention and resources, which could harm our business.

A significant portion of our total outstanding shares are restricted from resale but may be sold into the market in the near future. This could cause the trading price of our Common Stock to drop significantly, even if our business is doing well.

Sales of a substantial number of shares of our Common Stock in the public market could occur at any time. These sales, or the perception in the market that the holders of a large number of shares intend to sell shares, could reduce the trading price of our Common Stock. We had 32,948,332 shares of Common Stock outstanding as of March 27, 2024, of which (a) approximately 49.7% are subject to a 180-day lock-up period that began on November 2, 2023 and expires on April 30, 2024, provided under lock-up agreements executed in connection with our IPO and (b) approximately 0.4% are subject to a 90-day lock-up period that began on March 11, 2024 and expires on June 11, 2024, provided under lock-up agreements executed in connection with our Private Placement. All of these shares will, however, be able to be resold after the expiration of the lock-up period, as well as pursuant to customary exceptions thereto or upon the waiver of the lock-up agreement by or on behalf of the underwriters. We registered shares of Common Stock subject to options or other equity awards issued or reserved for future issuance under our equity compensation plans. Those shares can be freely sold in the public market upon issuance, subject to the lock-up agreements. As restrictions on resale end, the trading price of our stock could decline if the holders of currently-restricted shares sell them or are perceived by the market as intending to sell them.

If equity research analysts do not publish research or reports, or publish unfavorable research or reports, about us, our business or our market, our stock price and trading volume could decline.

The trading market for our Common Stock is influenced by the research and reports that equity research analysts publish about us and our business. As a newly public company, we have only limited research coverage by equity research analysts. Equity research analysts may elect not to provide research coverage of our Common Stock, and such lack of research coverage may adversely affect the trading price of our Common Stock. While we currently have equity research analyst coverage, we will not have any control over the analysts or the content and opinions included in their reports. The price of our stock could decline if one or more equity research analysts downgrade our stock or issue other unfavorable commentary or research. If one or more equity research analysts ceases coverage of our company or fails to publish reports on us regularly, demand for our stock could decrease, which in turn could cause our stock price or trading volume to decline.

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Our executive officers, directors and their affiliates, if they choose to act together, have the ability to significantly influence all matters submitted to stockholders for approval and may prevent new investors from influencing significant corporate decisions.

Based on the number of shares of Common Stock outstanding as of March 27, 2024, our executive officers, directors and stockholders who own more than 5% of our outstanding Common Stock and their respective affiliates beneficially hold, in the aggregate, shares representing approximately 38.9% of our outstanding Common Stock. As a result, if these stockholders choose to act together, they would be able to significantly influence all matters submitted to our stockholders for approval, as well as our management and affairs. For example, these persons, if they choose to act together, would significantly influence the election of directors, the composition of our management and approval of any merger, consolidation, sale of all or substantially all of our assets or other business combination that other stockholders may desire.

Some of these persons or entities may have interests different than yours. For example, because many of these stockholders purchased their shares at prices substantially below the current trading price of our Common Stock and have held their shares for a longer period, they may be more interested in selling our company to an acquirer than other investors, or they may want us to pursue strategies that deviate from the interests of other stockholders. Any of these actions could adversely affect the trading price of our Common Stock.

We are an “emerging growth company” and a “smaller reporting company” and, as a result of the reduced disclosure and governance requirements applicable to emerging growth companies and smaller reporting companies, our Common Stock may be less attractive to investors.

We are an “emerging growth company” as defined in the JOBS Act, and we intend to take advantage of some of the exemptions from reporting requirements that are applicable to other public companies that are not emerging growth companies, including:

- not being required to comply with the auditor attestation requirements in the assessment of our internal control over financial reporting;
- not being required to comply with any requirement that may be adopted by the Public Company Accounting Oversight Board regarding mandatory audit firm rotation or a supplement to the auditor’s report providing additional information about the audit and the financial statements;
- reduced disclosure obligations regarding executive compensation in our periodic reports, proxy statements and registration statements; and
- not being required to hold a nonbinding advisory vote on executive compensation and stockholder approval of any golden parachute payments not previously approved.

We cannot predict if investors will find our Common Stock less attractive because we will rely on these exemptions. If some investors find our Common Stock less attractive as a result, there may be a less active trading market for our Common Stock and our stock price may be more volatile. We may take advantage of these reporting exemptions until we are no longer an emerging growth company. We will remain an emerging growth company until the last day of the fiscal year ending after the fifth anniversary of our IPO, or, if earlier, (i) the last day of the fiscal year in which we have total annual gross revenue of at least \$1.235 billion, (ii) the date on which we are deemed to be a large accelerated filer, which means the market value of our Common Stock that is held by non-affiliates exceeds \$700 million as of the prior June 30, or (iii) the date on which we have issued more than \$1.0 billion in non-convertible debt during the prior three-year period.

In addition, we have elected to take advantage of the extended transition period to comply with new or revised accounting standards and to adopt certain of the reduced disclosure requirements available to emerging growth companies. As a result of the accounting standards election, we will not be subject to the same implementation timing for new or revised accounting standards as other public companies that are not emerging

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growth companies, which may make comparison of our financials to those of other public companies more difficult. As a result of these elections, the information that we provide in this prospectus may be different than the information investors may receive from other public companies in which they hold equity interests. In addition, it is possible that some investors will find our Common Stock less attractive as a result of these elections, which may result in a less active trading market for our Common Stock and higher volatility in our trading price.

Even after we no longer qualify as an emerging growth company, we may, under certain circumstances, still qualify as a “smaller reporting company,” which would allow us to take advantage of many of the same exemptions from disclosure requirements, including reduced disclosure obligations regarding executive compensation in our periodic reports and proxy statements.

Because we do not anticipate paying any cash dividends on our Common Stock in the foreseeable future, capital appreciation, if any, will be your sole source of gains and you may never receive a return on your investment.

You should not rely on an investment in our Common Stock to provide dividend income. We have not declared or paid cash dividends on our Common Stock to date. We currently intend to retain our future earnings, if any, to fund the development and growth of our business. As a result, capital appreciation, if any, of our Common Stock will be your sole source of gain for the foreseeable future. Investors seeking cash dividends should not purchase our Common Stock.

Our amended and restated certificate of incorporation provides that the Court of Chancery of the State of Delaware and the federal district courts of the United States of America will be the exclusive forums for substantially all disputes between us and our stockholders, which could limit our stockholders’ ability to obtain a favorable judicial forum for disputes with us or our directors, officers or employees.

Our amended and restated certificate of incorporation provides that the Court of Chancery of the State of Delaware is the exclusive forum for the following types of actions or proceedings under Delaware statutory or common law:

- any derivative action or proceeding brought on our behalf;
- any action asserting a breach of fiduciary duty;
- any action asserting a claim against us arising under the Delaware General Corporation Law, or DGCL, our amended and restated certificate of incorporation, or our amended and restated bylaws;
- any claim or cause of action seeking to interpret, apply, enforce or determine the validity of our restated certificate or our amended and restated bylaws;
- any claim or cause of action as to which the DGCL confers jurisdiction on the Court of Chancery of the state of Delaware; and
- any action asserting a claim against us that is governed by the internal-affairs doctrine.

This provision would not apply to suits brought to enforce a duty or liability created by the Securities Exchange Act of 1934, as amended, or the Exchange Act. Furthermore, Section 22 of the Securities Act creates concurrent jurisdiction for federal and state courts over all such Securities Act actions. Accordingly, both state and federal courts have jurisdiction to entertain such claims. To prevent having to litigate claims in multiple jurisdictions and the threat of inconsistent or contrary rulings by different courts, among other considerations, our amended and restated certificate of incorporation further provides that the federal district courts of the United States of America will be the exclusive forum for resolving any complaint asserting a cause of action arising under the Securities Act. While the Delaware courts have determined that such choice of forum provisions are facially valid, a stockholder may nevertheless seek to bring a claim in a venue other than those designated in the

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exclusive forum provisions. In such instance, we would expect to vigorously assert the validity and enforceability of the exclusive forum provisions of our amended and restated certificate of incorporation. This may require significant additional costs associated with resolving such action in other jurisdictions and there can be no assurance that the provisions will be enforced by a court in those other jurisdictions.

These exclusive forum provisions may result in increased costs for investors to bring a claim. Further, these exclusive forum provisions may limit a stockholder's ability to bring a claim in a judicial forum that it finds favorable for disputes with us or our directors, officers, or other employees, which may discourage lawsuits against us and our directors, officers and other employees. If a court were to find either exclusive forum provision in our amended and restated certificate of incorporation to be inapplicable or unenforceable in an action, we may incur further significant additional costs associated with resolving the dispute in other jurisdictions, all of which could seriously harm our business.

Provisions in our corporate charter documents and under Delaware law could make an acquisition of us, which may be beneficial to our stockholders, more difficult and may prevent attempts by our stockholders to replace or remove our current management.

Provisions in our certificate of incorporation and our bylaws may discourage, delay, or prevent a merger, acquisition, or other change in control of our company that stockholders may consider favorable, including transactions in which you might otherwise receive a premium for your shares. These provisions could also limit the price that investors might be willing to pay in the future for shares of our Common Stock, thereby depressing the trading price of our Common Stock. In addition, because our board of directors is responsible for appointing the members of our management team, these provisions may frustrate or prevent any attempts by our stockholders to replace or remove our current management by making it more difficult for stockholders to replace members of our board of directors. Among other things, these provisions:

- establish a classified board of directors such that only a portion of our directors stand for election at any given annual stockholder meeting;
- allow the authorized number of our directors to be changed from time to time by our shareholders or our board of directors;
- limit the manner in which stockholders can remove directors from our board of directors;
- establish requirements for stockholder proposals that can be acted on at stockholder meetings;
- require that stockholder actions must be effected at a duly called stockholder meeting and allow actions by our stockholders by written consent, with certain requirements;
- limit who may call stockholder meetings; and
- authorize our board of directors to issue preferred stock without stockholder approval, which could be used to institute a "poison pill" that would work to dilute the stock ownership of a potential hostile acquirer, effectively preventing acquisitions that have not been approved by our board of directors.

Moreover, because we are incorporated in Delaware, we are governed by the provisions of Section 203 of the DGCL, which prohibits a person who owns in excess of 15% of our outstanding voting stock from merging or combining with us for a period of three years after the date of the transaction in which the person acquired in excess of 15% of our outstanding voting stock, unless the merger or combination is approved in a prescribed manner.

General risks

Unstable market and economic conditions may have serious adverse consequences on our business and financial condition.

The global economy, including credit and financial markets, has experienced extreme volatility and disruptions recently, including, among other things, diminished liquidity and credit availability, declines in

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consumer confidence, declines in economic growth, supply chain shortages, increases in inflation rates, higher interest rates, and uncertainty about economic stability. For example, the COVID-19 pandemic resulted in widespread unemployment, economic slowdown and extreme volatility in the capital markets. The Federal Reserve has raised interest rates multiple times in response to concerns about inflation and it may raise them again. Higher interest rates, coupled with reduced government spending and volatility in financial markets, may increase economic uncertainty and affect consumer spending. Similarly, the ongoing military conflict between Russia and Ukraine and increasing tensions between China and Taiwan have created extreme volatility in the global capital markets and may have further global economic consequences, including disruptions of the global supply chain. Any such volatility and disruptions may adversely affect our business or the third parties on whom we rely. If the equity and credit markets deteriorate, including as a result of political unrest or war, it may make any necessary debt or equity financing more difficult to complete, more costly, and more dilutive. Failure to secure any necessary financing in a timely manner and on favorable terms could have a material adverse effect on our growth strategy, financial performance and share price and could require us to delay or abandon development or commercialization plans. In addition, there is a risk that one or more of our service providers, manufacturers or other partners would not survive or be able to meet their commitments to us under such circumstances, which could directly affect our ability to attain our operating goals on schedule and on budget. We have experienced and may in the future experience disruptions as a result of such macroeconomic conditions, including delays or difficulties in initiating or expanding clinical trials and manufacturing sufficient quantities of materials. Any one or a combination of these events could have a material and adverse effect on our results of operations and financial condition.

If we fail to maintain proper and effective internal controls, our ability to produce accurate financial statements on a timely basis could be impaired.

We are subject to the reporting requirements of the Exchange Act, the Sarbanes-Oxley Act of 2002, or the Sarbanes-Oxley Act, and the rules and regulations of the stock market on which our Common Stock is listed. The Sarbanes-Oxley Act requires, among other things, that we maintain effective disclosure controls and procedures and internal control over financial reporting.

Commencing with our fiscal year ending December 31, 2024, we must perform system and process evaluation and testing of our internal control over financial reporting to allow management to report on the effectiveness of our internal control over financial reporting in our Form 10-K filing for that year, as required by Section 404 of the Sarbanes-Oxley Act. This will require that we incur substantial additional professional fees and internal costs to expand our accounting and finance functions and that we expend significant management efforts. We have never been required to test our internal control within a specified period, and, as a result, we may experience difficulty in meeting these reporting requirements in a timely manner.

We may identify weaknesses in our system of internal financial and accounting controls and procedures that could result in a material misstatement of our financial statements. Our internal control over financial reporting will not prevent or detect all errors and all fraud. A control system, no matter how well designed and operated, can provide only reasonable, not absolute, assurance that the control system's objectives will be met. Because of the inherent limitations in all control systems, no evaluation of controls can provide absolute assurance that misstatements due to error or fraud will not occur or that all control issues and instances of fraud will be detected. If we fail to remediate our identified material weakness, or identify additional material weaknesses, in our internal control over financial reporting, if we are not able to comply with the requirements of Section 404 of the Sarbanes-Oxley Act in a timely manner, or if we are unable to maintain proper and effective internal controls, we may not be able to produce timely and accurate financial statements. If that were to happen, the trading price of our stock could decline and we could be subject to sanctions or investigations by the stock exchange on which our Common Stock is listed, the Securities and Exchange Commission, or the SEC, or other regulatory authorities.

Our ability to utilize our net operating loss carryforwards and research tax credits to offset future taxable income may be subject to limitations.

As of December 31, 2023, we had approximately \$70.2 million of U.S. federal net operating loss carryforwards, or NOLs, \$139.3 million of U.S. state and local NOLs, and \$7.1 million of federal tax credits. U.S. federal NOLs generated in taxable years beginning after December 31, 2017, do not expire and may be carried forward indefinitely, but the deductibility of such NOLs is limited to no more than 80% of current year taxable income. Our U.S. state and local NOLs begin to expire in 2040 and our federal research tax credits begin to expire in 2041.

In addition, under Sections 382 and 383 of the Internal Revenue Code of 1986, as amended, or the Code, if a corporation undergoes an “ownership change,” which is generally defined as a greater than 50% change, by value, in its equity ownership by certain stockholders over a rolling three-year period, the corporation’s ability to use its pre-change NOLs and certain other pre-change tax attributes (such as research tax credits) to offset its post-change taxable income or taxes may be limited. If we undergo an ownership change, and our ability to use our pre-change NOLs and other pre-change tax attributes (such as tax credits) to offset our post-change income or taxes is limited, it would harm our future results of operations by effectively increasing our future tax obligations. U.S. state and local NOLs may be similar limited. In addition, at the U.S. state and local level, there may be periods during which the use of NOLs is suspended or otherwise limited, which could accelerate or permanently increase U.S. state and local taxes owed.

Irrespective of the above, our ability to utilize our NOLs and research tax credits to offset future taxable income or taxes is conditioned on our attaining profitability and generating taxable income. We do not know if and when we will generate sufficient taxable income to utilize our NOLs and research tax credits.

Changes in tax laws or regulations that are applied adversely to us or our customers may materially harm our business.

New tax laws, statutes, rules, regulations, or ordinances could be enacted at any time. Further, existing tax laws, statutes, rules, regulations, or ordinances could be interpreted differently, changed, repealed, or modified at any time. Any such enactment, interpretation, change, repeal, or modification could adversely affect us, possibly with retroactive effect. The IRA enacted a 15% minimum tax on the adjusted financial statement income of certain large U.S. corporations for taxable years beginning after December 31, 2022, as well as a 1% excise tax on stock repurchases made by public corporations after December 31, 2022. Further, the Tax Cuts and Jobs Act of 2017, or the Tax Act, enacted many significant changes in U.S. federal tax laws, some of which were further modified by the Coronavirus Aid, Relief, and Economic Security Act, or the CARES Act, and may be modified in the future by the current or a future presidential administration. Among other changes, the Tax Act amended the Code to require that certain research and experimental expenditures be capitalized and amortized over five years if incurred in the United States or fifteen years if incurred in foreign jurisdictions for taxable years beginning after December 31, 2021. Although the U.S. Congress has considered legislation that would defer, modify, or repeal the capitalization and amortization requirement, there is no assurance that such changes will be made. If the requirement is not deferred, repealed, or otherwise modified, it may increase our cash taxes and effective tax rate. In addition, it is uncertain if and to what extent various states will conform to the IRA, the Tax Act, the CARES Act, or any future U.S. federal tax laws. Changes in corporate tax rates, the realization of net deferred tax assets relating to our operations, the taxation of foreign earnings, and the deductibility of expenses could have a material impact on the value of our deferred tax assets, result in significant one-time charges, and increase our future U.S. tax expenses.

Our business and operations would suffer in the event of system failures, cyberattacks or a deficiency in our or our CMOs’, CROs’, manufacturers’, contractors’, consultants’ or collaborators’ cybersecurity.

Despite the implementation of security measures, our internal computer systems, as well as those of third

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parties on which we rely, are vulnerable to damage from, among other things, computer viruses, malware, unauthorized access, natural disasters, terrorism, war telecommunication and electrical failures, system malfunctions, cyberattacks or cyber-intrusions over the Internet, attachments to emails, phishing attacks, persons inside our organization, or persons with access to systems inside our organization. The risk of a security breach or disruption, particularly through cyberattacks or cyber intrusion, including by computer hackers, foreign governments and cyber terrorists, has generally increased as the number, intensity and sophistication of attempted attacks and intrusions from around the world have increased. If such an event were to occur and cause interruptions in our operations, it could lead to the loss, destruction, alteration, prevention of access to, disclosure, dissemination of, or damage or unauthorized access to, our data (including trade secrets or other confidential information, intellectual property, proprietary business information and personal data) or data that is processed or maintained on our behalf, and cause interruptions in our operations, which could result in a material disruption of our product candidate development programs. For example, the loss of preclinical study or clinical trial data from completed, ongoing or planned trials could result in delays in our regulatory approval efforts and significantly increase our costs to recover or reproduce the data. To the extent that any disruption or security breach were to result in a loss of or damage to our data or applications, or inappropriate disclosure of personal, confidential or proprietary information, we could incur liability and the further development of our product candidates could be delayed.

In the ordinary course of our business, we collect and store sensitive data, including intellectual property, clinical trial data, proprietary business information, personal data and personally identifiable information of our clinical trial subjects and employees, in our data centers and on our networks. The secure processing, maintenance and transmission of this information is critical to our operations. Despite our security measures, we cannot ensure that our information technology and infrastructure will prevent breakdowns or breaches in our or their systems or other cybersecurity incidents that cause loss, destruction, unavailability, alteration, dissemination of, or damage or unauthorized access to, our data, including personal data, assets and other data processed or maintained on our behalf, that could have a material adverse effect upon our reputation, business, operations or financial condition. Although, to our knowledge, we have not experienced any such material security breach to date, any such breach could compromise our networks and the information stored there could be accessed, publicly disclosed, lost or stolen. Any such access, disclosure or other loss of information could result in legal claims or proceedings, liability under laws that protect the privacy of personal information, significant regulatory penalties, and such an event could disrupt our operations, damage our reputation, and cause a loss of confidence in us and our ability to conduct clinical trials, which could adversely affect our reputation and delay clinical development of our product candidates.

To the extent that any disruption or security breach were to result in a loss of or damage to our data or applications, or inappropriate disclosure of confidential or proprietary information or personal data, we could incur material legal claims and liability and damage to our reputation, and the further development of our product candidates could be delayed. Any such event could also compel us to comply with federal and state breach notification laws, and foreign law equivalents, subject us to mandatory corrective action and otherwise subject us to substantial liability under laws, rules, regulations and standards that protect the privacy and security of personal data, which could result in significant legal and financial exposure and reputational damages that could potentially have an adverse effect on our business.

Notifications and follow-up actions related to a data breach or other security incident could impact our reputation and cause us to incur significant costs, including significant legal expenses and remediation costs. We expect to incur significant costs in an effort to detect and prevent security incidents, and we may face increased costs and requirements to expend substantial resources in the event of an actual or perceived security incident. However, we cannot guarantee that we will be able to detect or prevent any such incidents, or that we can remediate any such incidents in an effective or timely manner. Our efforts to improve security and protect data from compromise may also identify previously undiscovered instances of data breaches or other cybersecurity incidents. To the extent that any data breach, disruption or security incident were to result in any loss, destruction, or alteration of, damage, unauthorized access to or inappropriate or unauthorized disclosure or

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dissemination of, our data, including personal data, or other information that is processed or maintained on our behalf, we could be exposed to litigation and governmental investigations and inquiries, the further development and commercialization of our product candidates could be delayed, and we could be subject to significant fines or penalties for any noncompliance with applicable state, federal and foreign privacy and security laws, rules, regulations and standards.

We incur increased costs and demands upon management as a result of being a public company.

As a public company listed in the United States, we incur significant additional legal, accounting and other expenses that we did not incur as a private company, including the cost of director and officer liability insurance. These additional costs could negatively affect our financial results. In addition, changing laws, regulations and standards relating to corporate governance and public disclosure, including regulations implemented by the SEC and the Nasdaq Stock Market, may increase legal and financial compliance costs and make some activities more time-consuming. These laws, regulations and standards are subject to varying interpretations and, as a result, their application in practice may evolve over time as new guidance is provided by regulatory and governing bodies. We intend to invest resources to comply with evolving laws, regulations and standards, and this investment may result in increased general and administrative expenses and a diversion of management's time and attention from revenue-generating activities to compliance activities. If notwithstanding our efforts to comply with new laws, regulations and standards, we fail to comply, regulatory authorities may initiate legal proceedings against us and our business may be harmed.

Failure to comply with these rules might also make it more difficult for us to obtain some types of insurance, including director and officer liability insurance, and we might be forced to accept reduced policy limits and coverage or incur substantially higher costs to obtain the same or similar coverage. The impact of these events could also make it more difficult for us to attract and retain qualified persons to serve on our board of directors, on committees of our board of directors or as members of senior management.

Our disclosure controls and procedures may not prevent or detect all errors or acts of fraud.

We are subject to certain reporting requirements of the Exchange Act. Our disclosure controls and procedures are designed to reasonably assure that information required to be disclosed by us in reports we file or submit under the Exchange Act is accumulated and communicated to management, recorded, processed, summarized, and reported within the time periods specified in the rules and forms of the SEC. We believe that any disclosure controls and procedures or internal controls and procedures, no matter how well conceived and operated, can provide only reasonable, not absolute, assurance that the objectives of the control system are met.

These inherent limitations include the realities that judgments in decision-making can be faulty, and that breakdowns can occur because of simple error or mistake. Additionally, controls can be circumvented by the individual acts of some persons, by collusion of two or more people, or by an unauthorized override of the controls. Accordingly, because of the inherent limitations in our control system, misstatements or insufficient disclosures due to error or fraud may occur and not be detected.

USE OF PROCEEDS

All of the Shares offered by the Selling Stockholders pursuant to this prospectus will be sold by the Selling Stockholders for their respective accounts. We will not receive any of the proceeds from the sale of the Shares hereunder.

**MARKET PRICE OF AND DIVIDENDS ON THE REGISTRANT'S COMMON EQUITY AND
RELATED STOCKHOLDER MATTERS**

Market Information and Holders

Our Common Stock trades on Nasdaq under the trading symbol "LXEO".

As of April 8, 2024, we had approximately 32,947,553 shares of Common Stock issued and outstanding held of record by 105 holders. We believe a greater number of holders of our Common Stock are "street name" or beneficial holders, whose shares of record are held by banks, brokers and other financial institutions.

Dividend Policy

We have never declared or paid, and do not anticipate declaring or paying, in the foreseeable future, any cash dividends on our capital stock. We currently intend to retain all available funds and any future earnings to support our operations and finance the growth and development of our business. Any future determination related to our dividend policy will be made at the discretion of our board of directors and will depend upon, among other factors, our results of operations, financial condition, capital requirements, contractual restrictions, business prospects and other factors our board of directors may deem relevant.

MANAGEMENT'S DISCUSSION AND ANALYSIS OF FINANCIAL CONDITION AND RESULTS OF OPERATIONS

You should read the following discussion and analysis of our financial condition and results of operations together with our consolidated financial statements and related notes included elsewhere in this prospectus. Some of the information contained in this discussion and analysis or set forth elsewhere in this prospectus, including information with respect to our plans and strategy for our business and related financing includes forward-looking statements that involve risks and uncertainties. Many factors, including those factors set forth in the "Risk Factors" section of this prospectus, may materially and adversely affect our actual results, which may differ materially from the results described in or implied by the forward-looking statements contained in the following discussion and analysis.

Overview

We are a clinical stage genetic medicine company dedicated to transforming healthcare by applying pioneering science to fundamentally change how disease is treated. By taking aim at the underlying genetic cause of the devastating diseases we target, we seek to create substantial positive impact and reduce the overwhelming burdens placed on people receiving treatment, their caregivers, and healthcare systems. Our current pipeline consists of candidates targeting patient populations that place significant burden on society and are most amenable to our genetic medicine approach.

Since our inception, we have devoted substantially all of our efforts to organizing and staffing our company, research and development activities, business planning, raising capital, building our intellectual property portfolio and providing general and administrative support for these operations. To date, we have funded our operations primarily through proceeds from the sale of shares of our convertible preferred stock and Common Stock, including our IPO and the subsequent partial exercise of the underwriters' 30-day option to purchase additional shares of Common Stock. As of December 31, 2023, we had \$121.5 million of cash and cash equivalents, and we had raised aggregate net proceeds of \$100.3 million from our IPO and the subsequent partial exercise of the underwriters' 30-day option to purchase additional shares of Common Stock, as well as totals of approximately \$88.9 million, \$185.0 million and \$3.9 million of net proceeds from the Private Placement (see Note 14 to the audited consolidated financial statements appearing elsewhere in this prospectus for more information), the sales of our convertible equity securities, and a convertible SAFE Note, respectively. We have incurred significant operating losses since the commencement of our operations. Our ability to generate product revenue sufficient to achieve profitability will depend heavily on the successful development and eventual commercialization of one or more of our current gene therapy candidates or any future gene therapy candidates. Our net losses for the years ended December 31, 2022 and December 31, 2023 were \$59.3 million and \$66.4 million, respectively, and our accumulated deficit was \$181.8 million at December 31, 2023. We expect to continue to incur significant losses for the foreseeable future as we advance our current and future product candidates through preclinical and clinical development, continue to build our operations and transition to operating as a public company.

We expect to continue to incur net operating losses for at least the next several years, and we expect our research and development expenses, general and administrative expenses, and capital expenditures to continue to increase. We expect our expenses and capital requirements will increase significantly in connection with our ongoing activities as we:

- continue our ongoing and planned clinical trials as well as research and development of our Friedreich's ataxia cardiomyopathy, or FA cardiomyopathy (LX2006), APOE-associated Alzheimer's disease (LX1001), and arrhythmogenic cardiomyopathy caused by mutations in the *PKP2* gene, or PKP2-ACM (LX2020) programs and other product candidates;
- initiate preclinical studies and clinical trials for any additional product candidates that we may pursue in the future;

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- seek to discover and develop additional product candidates and further expand our clinical product pipeline;
- seek regulatory approvals for any product candidates that successfully complete clinical trials;
- invest in capital equipment in order to expand our research and development and manufacturing activities;
- attract, hire and retain additional clinical, scientific, quality control, regulatory, and manufacturing management and administrative personnel;
- add clinical, operational, financial and management information systems and personnel, including personnel to support our product development;
- develop, maintain, expand, protect and enforce our intellectual property portfolio, including patents, trade secrets and know-how;
- acquire or in-license other product candidates and technologies;
- expand our operations in the United States and to other geographies;
- incur additional legal, accounting, investor relations and other general and administrative expenses associated with operating as a public company; and
- establish a sales, marketing and distribution infrastructure, either ourselves or in partnership with others, to commercialize any product candidates, if approved, and related additional commercial manufacturing costs.

Summary of key factors impacting the comparability of our performance

Stelios acquisition

On July 16, 2021, we purchased all of the issued and outstanding capital stock of Stelios for initial cash consideration of \$7.0 million, with payments of up to an additional \$20.5 million due upon the achievement of the following milestones: clinical candidate selection for either the ACM or the TNNI3-associated programs; first patient dosed in a Phase 1 clinical trial for any product in our Cx43 program; first patient dosed in a Phase 1 clinical trial for any product in our ACM program; or first patient dosed in a Phase 1 clinical trial for any candidate in our *TNNI3* program. In the third quarter of 2022, a development milestone associated with a clinical candidate selection was achieved resulting in a \$2.0 million payment to the selling shareholders of Stelios.

We accounted for the acquisition of Stelios as an asset acquisition pursuant to Financial Accounting Standards Board, or FASB, Accounting Standards Codification, or ASC, Section 805, *Business Combinations*. We acquired in-process research and development, or IPR&D, assets from Stelios related to ACM and TNNI3-associated hypertrophic cardiomyopathy programs. Such programs relate to our development efforts for LX2020, LX2021 and LX2022. The fair value of the IPR&D acquired of \$7.0 million was charged to research and development expense as it had no alternative future use at the time of the acquisition. Stelios was merged into Lexeo Therapeutics, Inc. on December 15, 2022 and ceased to exist.

See Note 13 to the audited consolidated financial statements appearing elsewhere in this prospectus for more information on the Stelios acquisition.

Lease agreement

In February 2022, we entered into a lease agreement for our New York headquarters located at 345 Park Avenue South, 6th Floor, New York, NY 10010, pursuant to which we lease a total of approximately 15,839 square feet of office and laboratory space. The lease began in April 2022 and ends in July 2029 with an additional five-year option to extend at the then prevailing effective market rental rate. We accounted for this

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lease pursuant to FASB ASC Topic 842, *Leases*. Upon commencement of this lease, we recorded operating lease right-of-use assets and operating lease liabilities of approximately \$11.6 million based on the present value of payments over the lease term using an estimated incremental borrowing rate of 8.53%. In connection with this lease, we provided a security deposit to the landlord in the form of a letter of credit totaling \$1.2 million.

See Note 5 to the audited consolidated financial statements appearing elsewhere in this prospectus for more information on this lease.

Components of our results of operations

Revenue

Our revenue to date has been comprised of grant revenue, which are amounts earned from performing contracted research and development services. These grants generally require us to meet certain research milestones in order to receive the funds. To date, we have not generated any revenue from product sales. If our development efforts for LX2006, LX1001, and LX2020 or any future product candidates, are successful and result in regulatory approval, or if we enter into collaboration or license agreements with third parties, we may generate revenue in the future from product sales, royalties or payments from such collaboration or license agreements, or a combination of product sales and payments from such agreements.

Operating expenses

Research and development

Research and development expenses consist of costs incurred for our research activities, including our discovery efforts and the preclinical and clinical development of our programs. These expenses include:

- employee-related expenses, including salaries, benefits, and stock-based compensation expense for employees engaged in research and development functions;
- expenses incurred under agreements with third parties, such as consultants, clinical investigators, contractors and CROs that assist with (i) identification of potential product candidates in discovery platforms and (ii) the preclinical and clinical studies of our product candidates;
- the cost of developing and scaling our manufacturing process and manufacturing product candidates for use in our research, preclinical studies and clinical trials, including under agreements with third parties, such as consultants, contractors and CMOs;
- costs to maintain compliance with FDA and other regulatory requirements;
- laboratory supplies and research materials;
- facilities, depreciation, and other expenses, which include direct and allocated expenses for rent and maintenance of facilities;
- payments made under our licensing agreements with third parties, including milestone payments; and
- other expenses incurred as a result of research and development activities.

We expense research and development costs as incurred. Nonrefundable advance payments for goods and services that will be used in future research and development activities are expensed when the activity has been performed or when the goods have been received rather than when the payment is made. When third-party service providers' billing terms do not coincide with our period-end, we are required to make estimates of our obligations to those third parties incurred in a given accounting period and record accruals at the end of the period. We base these estimates on our knowledge of the research and development programs, services performed for the period, past history for related activities and the expected duration of the third-party service contract, where applicable. If timelines or contracts are modified based upon changes in the scope of work to be

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performed, we modify our estimates of accrued expenses accordingly on a prospective basis; therefore, actual results could differ from our estimates. Upfront payments under license agreements are expensed upon receipt of the license, and annual maintenance fees under license agreements are expensed in the period in which they are incurred. Milestone payments under license agreements are accrued, with a corresponding expense being recognized, in the period in which the milestone is determined to be probable of achievement and the related amount is reasonably estimable.

Our direct research and development expenses are tracked on a program-by-program basis and consist primarily of external costs, such as fees paid to CROs, CMOs, central laboratories and certain outside consultants in connection with our research and discovery, preclinical development, process development, manufacturing, clinical development, clinical trials, regulatory and quality assurance activities. We do not allocate professional services costs and licensing fees and other similar costs to specific programs because these costs are deployed across multiple programs.

Research and development activities are central to our business model and account for a significant portion of our operating expenses. Product candidates in later stages of clinical development generally have higher development costs than those in earlier stages of clinical development, primarily due to the increased size and duration of later-stage clinical trials. As a result, we expect our research and development expenses to increase substantially in absolute dollars for the foreseeable future as we further advance LX2006, LX2020, LX1001, and any other future product candidates that we may develop, into and through preclinical studies and clinical trials and pursue regulatory approvals. We cannot determine with certainty the timing of initiation, the duration or the completion costs of current or future preclinical studies and clinical trials of our product candidates due to the inherently unpredictable nature of preclinical and clinical development. Clinical and preclinical development timelines, the probability of success and development costs can differ materially from expectations. We anticipate that we will make determinations as to which product candidates to pursue and how much funding to direct to each product candidate on an ongoing basis in response to the results of ongoing and future research, preclinical studies and clinical trials, regulatory developments and our assessments as to each product candidate's commercial potential. In addition, we cannot forecast whether any of our current or future product candidates will be subject to future collaborations, when such arrangements will be secured, if at all, and to what degree such arrangements would affect our development plans and capital requirements. We are also unable to predict when, if ever, we will generate revenue from our product candidates to offset our expenses.

General and administrative

General and administrative expenses consist primarily of personnel expenses, including salaries, benefits and stock-based compensation expense, for personnel in executive, accounting, business development, legal, human resources and administrative functions. General and administrative expenses also include corporate facility costs not otherwise included in research and development expenses, depreciation and other expenses, which include direct or allocated expenses for rent and maintenance of facilities and insurance, not otherwise included in research and development expenses, as well as professional fees for legal, consulting, investor and public relations, accounting and audit services.

We expect that our general and administrative expenses will increase significantly in the near-to-medium term as we incur additional expenses associated with operating as a public company, including increased expenses for insurance premiums and audit, legal, regulatory, and tax-related services associated with maintaining compliance with the rules and regulations of the SEC and standards applicable to companies listed on a national securities exchange, investor relations activities and other administrative and professional services. We also plan to increase our general and administrative headcount to support the continued research and development of our programs and the growth of our business.

Fair value adjustment to convertible SAFE Note

Fair value adjustment to convertible SAFE Note consists of gains (losses) on the estimated fair value of the convertible SAFE Note.

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Other income (expense)

Other income (expense) includes net foreign exchange gains (losses).

Interest expense

Interest expense is primarily associated with our finance right of use asset equipment leases, as well as amortization of the discount to our convertible SAFE Note liability balance since August 2023.

Interest income

Interest income is primarily related to interest earned from our investment in a U.S. government money market fund, as well as interest earned on interest-bearing demand deposit cash accounts.

Income taxes

Provision for income taxes consists of U.S. federal and state income taxes in which we conduct business. Since our inception, we have not recorded any income tax benefits for the net losses we have incurred in each year or for our research and development tax credits, as we believe, based upon the weight of available evidence, that it is more likely than not that all of our NOL carryforwards and tax credits will not be realized. Accordingly, we have recorded a full valuation allowance against our net deferred tax assets at December 31, 2023, and December 31, 2022. As of December 31, 2023 and December 31, 2022 we had no unrecognized tax benefits.

Results of operations

Comparison of the Years Ended December 31, 2023 and 2022

The following table summarizes our results of operations for the years ended December 31, 2023 and 2022 (in thousands):

	Year Ended December 31,		
	2023	2022	Change
Revenue			
Grant revenue	\$ —	\$ 654	\$ (654)
Total revenue	—	654	(654)
Operating expenses			
Research and development	53,130	49,162	3,968
General and administrative	15,383	12,001	3,382
Total operating expenses	68,513	61,163	7,350
Operating loss	(68,513)	(60,509)	(8,004)
Other income and expense			
Loss on fair value adjustment to convertible SAFE Note	(530)	—	(530)
Other income (expense), net	(13)	(2)	(11)
Interest expense	(205)	(91)	(114)
Interest income	2,867	1,325	1,542
Total other income and expense	2,119	1,232	887
Loss from operations before income taxes	(66,394)	(59,277)	(7,117)
Income taxes	—	—	—
Net loss and comprehensive loss	\$(66,394)	\$(59,277)	\$(7,117)

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Grant revenue

We did not record any grant revenue for the year ended December 31, 2023, and grant revenue was \$0.7 million for the year ended December 31, 2022.

The decrease in grant revenue was due to the wind-down of the underlying grants, which was completed in the third quarter of 2022.

Research and development expenses

The following table summarizes our research and development expenses incurred for the years ended December 31, 2023 and 2022 (in thousands):

	Year Ended December 31,		
	2023	2022	Change
Direct external research and development expenses by program:			
LX2020	\$14,192	\$ 8,551	\$ 5,641
LX1001	9,936	7,605	2,331
LX2006	7,537	7,591	(54)
LX1004	310	1,783	(1,473)
Other programs	2,214	5,226	(3,012)
Total direct external research and development expenses by program	34,189	30,756	3,433
Unallocated research and development expenses:			
Employee compensation and stock-based compensation expenses	13,499	10,087	3,412
Professional fees	1,707	1,341	366
Lab-related costs and supplies	1,103	979	124
License fees	6	451	(445)
Other unallocated	2,626	5,548	(2,922)
Total unallocated research and development expenses	18,941	18,406	535
Total research and development expenses	\$53,130	\$49,162	\$ 3,968

The net increase of \$4.0 million in total research and development expenses for the year ended December 31, 2023 compared to the year ended December 31, 2022 was primarily due to increases in (i) clinical trial costs of \$5.3 million, (ii) employee compensation and stock-based compensation expenses of \$3.4 million due to increased headcount, and (iii) net milestone expense of \$1.5 million that consisted of a \$3.5 million milestone paid to Adverum in 2023 that was partially offset by a \$2.0 million milestone payment made to the selling shareholders of Stelios during the year ended December 31, 2022. These increases were partially offset by decreases in (i) non-clinical and preclinical expenses of \$5.4 million primarily related to our early stage cardiovascular and CNS disease programs, (ii) other unallocated expenses of \$2.9 million, and (iii) CMC expenses of \$0.9 million.

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General and administrative expenses

General and administrative expenses were \$15.4 million for the year ended December 31, 2023 compared to \$12.0 million for the year ended December 31, 2022. The increase of \$3.4 million was primarily due to increases in (i) \$1.9 million of employee compensation and stock-based compensation expenses due to increased headcount, (ii) \$1.7 million of third-party legal costs, and (iii) \$0.3 million of rent expense as the operating lease for our New York headquarters commenced in April 2022, which were partially offset by a decrease of \$0.4 million in third-party audit, accounting service provider, and professional service fees.

Interest income

We recognized interest income of \$2.9 million and \$1.3 million for the years ended December 31, 2023 and December 31, 2022, respectively, primarily related to interest earned on our investment in a U.S. government money market fund, with increased interest rates on such money market fund investment earned over those periods.

Liquidity and capital resources

Sources of liquidity

Since our inception, we have not generated any revenue from product sales and have incurred significant operating losses and negative cash flows from our operations. We expect to incur significant expenses and operating losses for the foreseeable future as we advance the clinical development of our product candidates. Since our inception through December 31, 2023, we have funded our operations primarily with net proceeds from sales of our convertible preferred stock, convertible SAFE Note, and shares of Common Stock sold in our IPO and the subsequent partial exercise of the underwriters' 30-day option to purchase additional shares of Common Stock collectively totaling \$289.1 million. As of December 31, 2023, we had cash and cash equivalents of \$121.5 million.

Based on our current operating plans, we expect that our existing cash and cash equivalents and the gross proceeds of \$95.0 million received upon the closing of the Private Placement will be sufficient to fund our planned operating expenses and capital expenditure requirements into 2027. Our total future capital requirements will depend on many factors and is subject to the risks and uncertainties set forth in the section titled "*Risk Factors*."

Cash flows

The following table summarizes our sources and uses of cash for the years ended December 31, 2023 and December 31, 2022 (in thousands):

	<u>Year Ended December 31,</u>	
	<u>2023</u>	<u>2022</u>
Net cash used in operating activities	\$ (59,496)	\$ (54,560)
Net cash used in investing activities	(165)	(901)
Net cash provided by financing activities	103,791	189
Net increase (decrease) in cash	<u>\$ 44,130</u>	<u>\$ (55,272)</u>

Operating activities

During the year ended December 31, 2023, net cash used in operating activities consisted primarily of our net loss of \$66.4 million, which was partially offset by (i) \$3.0 million of stock-based compensation expense, (ii) \$1.6 million of amortization of our right-of-use assets for our operating and finance leases, and (iii) \$1.5 million of net cash provided by changes in operating assets and liabilities.

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During the year ended December 31, 2022, net cash used in operating activities consisted primarily of our net loss of \$59.3 million, which was partially offset by (i) \$1.8 million of net cash provided by changes in operating assets and liabilities, (ii) \$1.8 million of stock-based compensation expense, and (iii) \$1.0 million of amortization of our right-of-use assets for our operating and finance leases.

Investing activities

During the years ended December 31, 2023 and 2022, net cash used in investing activities was \$0.2 million and \$0.9 million, respectively, and consisted of the purchase of lab equipment and internal use software.

Financing activities

During the year ended December 31, 2023, net cash provided by financing activities consisted primarily of the net proceeds received from the IPO of \$100.3 million, as well as net proceeds received from the issuance of the convertible SAFE Note of \$3.9 million, which were partially offset by \$0.4 million of principal payments made on equipment finance leases.

During the year ended December 31, 2022, net cash provided by financing activities consisted primarily of proceeds received from the exercise of stock options of \$0.3 million, which was partially offset by \$0.2 million of principal payments made on equipment finance leases.

Our primary uses of cash are to fund our clinical trials, research and development activities related to our discovery programs and our preclinical and clinical product candidates, hiring personnel, raising capital and providing general and administrative support for these operations.

Funding requirements

We expect our expenses and capital requirements to increase significantly in connection with our ongoing activities, particularly as we advance our lead product candidates and other development programs. Further, we incur costs associated with operating as a public company. Accordingly, and beyond the net proceeds raised in the IPO and Private Placement, we will require substantial additional funding to support our continuing operations.

The timing and amount of our future operating and capital requirements will largely depend on many factors, including:

- the initiation, scope, progress, timing, results and costs of product discovery, preclinical studies and clinical trials for our product candidates or any future candidates we may develop;
- our ability to maintain our relationships with Weill Cornell Medicine, Adverum, UCSD, and any other key licensors or collaborators;
- the scope, prioritization and number of our research and development programs;
- the costs, timing and outcome of seeking and obtaining regulatory approvals from the FDA and comparable foreign regulatory authorities, including the potential for such authorities to require that we perform more preclinical studies or clinical trials than those that we currently expect or change their requirements on studies that had previously been agreed to;
- our ability to establish and maintain collaborations on favorable terms, if at all;
- the achievement of milestones or occurrence of other developments that trigger payments under any collaboration agreements we have or may enter into;
- the extent to which we are obligated to reimburse, or entitled to reimbursement of, clinical trial costs under collaboration agreements, if any;

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- the costs to establish, maintain, expand, enforce and defend the scope of our intellectual property portfolio, including preparing, filing and prosecuting patent applications, maintaining and enforcing our intellectual property rights and defending intellectual property-related claims;
- the extent to which we acquire or in-license other product candidates and technologies;
- the costs of securing manufacturing arrangements for commercial production;
- the costs of establishing or contracting for sales and marketing capabilities if we obtain regulatory approvals to market our product candidates; and
- our need to implement additional internal systems and infrastructure.

We may be unable to raise additional funds or enter into potential collaborations, strategic partnerships or marketing, distribution, licensing or other similar agreements or arrangements on favorable terms, or at all. To the extent that we raise additional capital through the sale of equity or convertible debt securities, your ownership interest will be diluted. If we fail to raise capital or enter into such agreements or arrangements as, and when, needed, we may have to significantly delay, scale back or discontinue the development or commercialization of our product candidates or grant rights to develop and market product candidates that we would otherwise prefer to develop and market ourselves.

Critical accounting policies and significant judgments and estimates

Our management's discussion and analysis of our financial condition and results of operations are based on our consolidated financial statements, which have been prepared in accordance with U.S. generally accepted accounting principles, or U.S. GAAP. The preparation of our consolidated financial statements and related disclosures requires us to make estimates and judgments that affect the reported amounts of assets, liabilities, expenses, and the disclosure of our contingent liabilities in our consolidated financial statements. We base our estimates on historical experience, known trends and events and various other factors that we believe are reasonable under the circumstances, the results of which form the basis for making judgments about the carrying values of assets and liabilities that are not readily apparent from other sources. We evaluate our estimates and assumptions on an ongoing basis. Our actual results may differ from these estimates under different assumptions or conditions.

While our significant accounting policies are described in more detail in Note 2 to our audited consolidated financial statements included elsewhere in this prospectus, we believe that the following accounting policies are those most critical to the judgments and estimates used in the preparation of our audited consolidated financial statements.

Research and development

As part of the process of preparing our consolidated financial statements, we are required to estimate our accrued research and development expenses. This process involves estimating the level of service performed and the associated cost incurred for the service when we have not yet been invoiced or otherwise notified of actual costs. The majority of our service providers invoice us in arrears for services performed, on a pre-determined schedule or when contractual milestones are met; however, some require advance payments. We make estimates of our accrued expenses as of each balance sheet date in the consolidated financial statements based on facts and circumstances known to us at that time. At each period end, we corroborate the accuracy of these estimates with the service providers and make adjustments, if necessary. Examples of estimated accrued research and development expenses include those related to fees paid to:

- vendors in connection with discovery and preclinical development activities;
- CROs in connection with preclinical studies and testing; and

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- CMOs in connection with the process development and scale up activities and the production of materials.

We record the expense and accrual related to contract research and manufacturing based on our estimates of the services received and efforts expended considering a number of factors, including our knowledge of the progress towards completion of the research, development, and manufacturing activities; invoicing to date under contracts; communication from the CROs, CMOs, and other companies of any actual costs incurred during the period that have not yet been invoiced; and the costs included in the contracts and purchase orders. The financial terms of these agreements are subject to negotiation, vary from contract to contract, and may result in uneven payment flows. There may be instances in which payments made to our vendors will exceed the level of services provided and result in a prepayment of the expense. In accruing service fees, we estimate the time period over which services will be performed and the level of effort to be expended in each period. If the actual timing of the performance of services or the level of effort varies from the estimate, we adjust the accrual or the amount of prepaid expense accordingly. Although we do not expect our estimates to be materially different from amounts actually incurred, our understanding of the status and timing of services performed relative to the actual status and timing of services performed may vary and may result in reporting amounts that are too high or too low in any particular period. To date, there have not been any material adjustments to our prior estimates of accrued research and development expenses; however, we cannot guarantee that such adjustments will not be made in the future.

Determination of fair value of Common Stock

Given the lack of an active public market for our Common Stock and other equity instruments prior to our IPO, the estimated fair value of our Common Stock was determined by our board of directors as of the date of each option grant, with input from management, considering our most recently available third-party valuations of Common Stock and our board of directors' assessment of additional objective and subjective factors that it believed were relevant and which may have changed from the date of the most recent valuation through the date of the grant. These third-party valuations were performed in accordance with the guidance outlined in the American Institute of Certified Public Accountants' Accounting and Valuation Guide, Valuation of Privately-Held-Company Equity Securities Issued as Compensation.

Our Common Stock valuations were prepared using either an option pricing method, or OPM, or a hybrid method of OPM and probability-weighted expected return method, or PWERM. Both the OPM and hybrid methods use market approaches to estimate our enterprise value. These third-party valuations were performed at various dates, which resulted in valuations of our Common Stock of \$14.62 as of December 17, 2021, \$15.15 as of February 15, 2022, \$17.59 as of February 14, 2023, and \$11.02 as of June 23, 2023. In addition to considering the results of these third-party valuations, our board of directors considered various objective and subjective factors to determine the fair value of our Common Stock as of each grant date, including:

- the prices at which we sold shares of preferred stock and the superior rights and preferences of the preferred stock relative to our Common Stock at the time of each grant;
- the progress of our research and development programs, including the status and results of preclinical studies for our product candidates;
- our stage of development and our business strategy;
- external market conditions affecting the biopharmaceutical industry and trends within the biopharmaceutical industry;
- our financial position, including cash on hand, and our historical and forecasted performance and operating results;
- the lack of an active public market for our Common Stock and our preferred stock;

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- significant changes to the key assumptions underlying the factors used could have resulted in different fair values of Common Stock at each valuation date; the likelihood of achieving a liquidity event, such as an IPO, or sale of our company in light of prevailing market conditions; and
- the analysis of IPOs and the market performance of similar companies in the therapeutics industry.

Following the closing of our IPO, the fair value of our Common Stock is determined based on the quoted market price of our Common Stock on the date of grant.

Stock option awards

We recognize share-based compensation expense related to stock option awards granted based on the estimated fair value of the awards on the date of grant using a Black-Scholes option pricing model. The grant date fair value of the share-based awards is recognized on a straight-line basis over the requisite service period, which is the vesting period of the respective awards. Share-based payments to non-employees issued in exchange for services are based upon the fair value of the equity instruments issued. Compensation expense for stock options issued to non-employees is calculated using the Black-Scholes option pricing model and is recorded over the requisite service performance period.

The Black-Scholes option-pricing model requires the use of highly subjective assumptions, which are used to determine the fair value stock option awards granted. These assumptions include:

Expected Term. The expected term represents the period that our stock option awards granted are expected to be outstanding and is determined using the simplified method (based on the mid-point between the vesting date and the end of the contractual term).

Expected Volatility. Because we did not have any trading history for our shares of Common Stock prior to the IPO, the expected volatility for stock options granted prior to the IPO was estimated entirely using averages of the historical volatility of our peer group of companies, and the expected stock price volatility was primarily based on the historical volatility of a publicly traded set of peer companies for stock options granted since the IPO, each for a period equal to the expected life of the stock options granted, and we expect to continue to do so until such time as we have adequate historical data regarding the volatility of our own traded stock price. Our peer group of publicly traded companies was chosen based on their similar size, stage in the life cycle or area of specialty.

Risk-Free Interest Rate. The risk-free interest rate is based on the interest rates paid on securities issued by the U.S. Treasury with a term approximating the expected life of stock options granted.

Expected Dividend. We have never paid, and do not anticipate paying, cash dividends on our shares of Common Stock. Therefore, the expected dividend yield was assumed to be zero.

Fair Value of Common Stock. Since our IPO, the fair value of shares of our Common Stock is measured by the stock price on the date of grant.

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The following table summarizes option activity for the year ended December 31, 2023:

<u>Grant Date</u>	<u>Number of Shares Subject to Options Granted</u>	<u>Per Share Exercise Price of Options</u>	<u>Per Share Fair Value of Common Stock on Grant Date</u>	<u>Per Share Estimated Fair Value of Options</u>
March 14, 2023	464,613	\$ 17.59	\$ 17.59	\$ 13.06
July 24, 2023	146,234	\$ 11.02	\$ 11.02	\$ 7.08
August 22, 2023	459,800	\$ 11.02	\$ 11.02	\$ 8.27
September 17, 2023	63,665	\$ 11.02	\$ 11.02	\$ 8.22
November 30, 2023	93,642	\$ 12.72	\$ 12.72	\$ 9.43

Emerging growth company status

The JOBS Act permits an “emerging growth company” such as us to take advantage of an extended transition period to comply with new or revised accounting standards applicable to public companies until those standards would otherwise apply to private companies. We have elected not to “opt out” of such extended transition period, which means that when a standard is issued or revised and it has different application dates for public or private companies, we will adopt the new or revised standard at the time private companies adopt the new or revised standard and will do so until such time that we either (i) irrevocably elect to “opt out” of such extended transition period or (ii) no longer qualify as an emerging growth company. There are other exemptions and reduced reporting requirements provided by the JOBS Act that we are currently evaluating. For example, as an “emerging growth company,” we are exempt from Sections 14A(a) and (b) of the Exchange Act, which would otherwise require us to (1) submit certain executive compensation matters to shareholder advisory votes, such as “say-on-pay,” “say-on-frequency,” and “golden parachutes;” and (2) disclose certain executive compensation related items such as the correlation between executive compensation and performance and comparisons of our chief executive officer’s compensation to our median employee compensation. We also rely on an exemption from the rule requiring us to provide an auditor’s attestation report on our internal controls over financial reporting pursuant to Section 404(b) of the Sarbanes-Oxley Act. We will continue to remain an “emerging growth company” until the earliest of the following: (1) the last day of the fiscal year following the fifth anniversary of the date of the completion of our IPO; (2) the last day of the fiscal year in which our total annual gross revenue is equal to or more than \$1.235 billion; (3) the date on which we have issued more than \$1.0 billion in nonconvertible debt during the previous three years; or (4) the date on which we are deemed to be a large accelerated filer under the rules of the SEC.

Recently issued accounting pronouncements

A description of recently issued accounting pronouncements that may potentially impact our financial position, results of operations and cash flows is disclosed in Note 2 to our audited consolidated financial statements included elsewhere in this prospectus.

Qualitative and quantitative disclosures about market risk

Interest rate risk

We are a smaller reporting company, as defined by Rule 12b-2 under the Exchange Act and are not required to provide qualitative and quantitative disclosures about market risk.

BUSINESS

We are a clinical stage genetic medicine company dedicated to transforming healthcare by applying pioneering science to fundamentally change how disease is treated. By taking aim at the underlying genetic cause of the devastating diseases we target, we seek to create substantial positive impact and reduce the overwhelming burdens placed on people receiving treatment, their caregivers, and healthcare systems. Our current pipeline consists of candidates targeting patient populations that place significant burden on society and are most amenable to our genetic medicine approach.

Our most advanced cardiovascular product candidate, LX2006 for the treatment of patients with Friedreich's ataxia, or FA, cardiomyopathy, is currently being evaluated in SUNRISE-FA, an ongoing Phase 1/2 clinical trial. We have observed an increase in frataxin protein expression in the hearts of three patients that have undergone cardiac biopsies across cohort 1 (n=1) and cohort 2 (n=2), and we expect to report additional interim data from this trial in mid-2024. Our second most advanced cardiovascular product candidate, LX2020 for the treatment of arrhythmogenic cardiomyopathy, or ACM, caused by mutations in the *PKP2* gene, referred to as PKP2-ACM, received IND clearance from the U.S. Food and Drug Administration, or FDA, in July 2023. LX2020 received Fast Track and Orphan Drug designations from the FDA in December 2023. We expect to dose the first patient in a Phase 1/2 clinical trial in the first half of 2024 and provide an interim data readout from cohort 1 in the second half of 2024.

Our lead Alzheimer's disease product candidate, LX1001 for the treatment of *APOE4* homozygous patients with Alzheimer's disease, is in an ongoing Phase 1/2 trial. In December 2022, we reported that we observed an increase in expression levels of the protective protein, *APOE2*, in the first dose cohort and a consistent trend towards improvement in core Alzheimer's disease biomarkers in the first dose cohort. We completed enrollment of the trial in the fourth quarter of 2023 and expect to report additional interim data from all cohorts in the Phase 1/2 clinical trial in the second half of 2024.

We are targeting diseases that have seen limited penetration of precision medicine, which we define as medications that treat the underlying molecular mechanism of a disease, and where we believe there is a significant opportunity for gene therapy to play a role as a key therapeutic option. We believe the specific indications we are initially targeting, FA cardiomyopathy, PKP2-ACM and *APOE4*-associated Alzheimer's disease are highly amenable to gene therapy, where administration of a single dose has the potential to either restore loss-of-function or minimize gain-of-function mutations by treating the underlying genetic cause of the disease. Although few precision medicines are currently approved for the treatment of cardiovascular conditions or Alzheimer's disease, recent approvals by the FDA suggest a willingness to approve new precision medicines based on biomarkers and functional endpoints. Together with improved diagnostics and increased testing, these developments may offer one of the most substantial opportunities for the uptake of precision medicines in the global pharmaceutical marketplace.

Each of our gene therapy candidates utilizes the vector construct, dose and route of administration that we believe will result in the most favorable biodistribution and safety profile for our product candidate for each disease. Our most advanced cardiovascular and *APOE4*-associated Alzheimer's disease programs use the AAVrh10 vector due to its high transduction efficiency in both myocardial cells and neurons, potential for lower toxicity given the opportunity to utilize lower doses compared to other well-established AAV serotypes, and low pre-existing immunity.

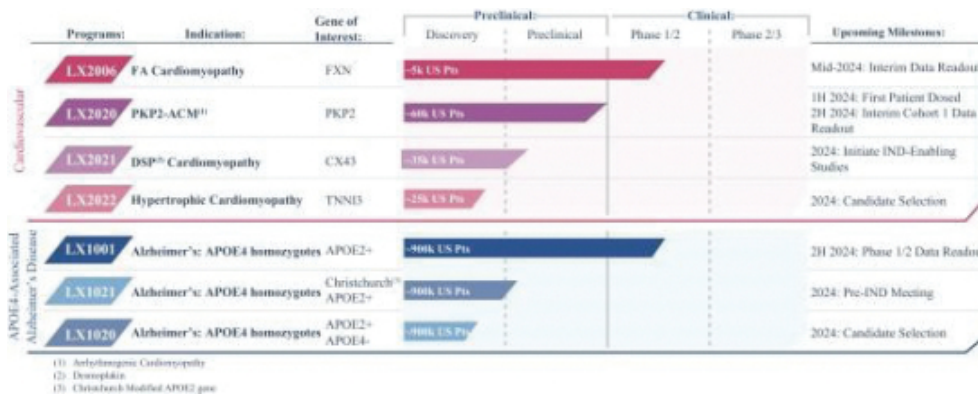
By specifically tailoring our technological approach to each targeted disease, we believe we can optimize our programs to achieve the highest likelihood of having therapeutic impact. We target genetically defined indications in specific sub-groups of patients that offer the potential to demonstrate therapeutic impact through improvement in functional endpoints or biomarkers, have high unmet need and large market opportunities, have established proof-of-concept in relevant preclinical models, and have organized patient advocacy groups and identifiable patient populations. In addition to targeting cardiovascular diseases and *APOE4*-associated

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Alzheimer’s disease that we believe can be addressed by our current approach utilizing AAVrh10, we have ongoing discovery efforts to identify next-generation vector technologies with the best potential therapeutic profile. Finally, we continuously seek to bolster our pipeline through relationships with academic institutions, providing us access to cutting edge genetic medicines research which will include not only AAV gene therapy but also other potential therapeutic payload types and non-viral delivery systems. In August 2023, we announced a strategic investment from Sarepta Therapeutics, Inc. to explore collaboration opportunities within our preclinical cardiovascular pipeline.

Our pipeline

Utilizing a stepwise, capital-efficient development approach, we are leveraging early proof-of-concept functional and biomarker data to advance a deep and diverse pipeline of cardiovascular and APOE4-associated Alzheimer’s disease programs for larger-rare and prevalent indications. We retain exclusive worldwide development and commercialization rights to all of our product candidates and programs.



Cardiovascular programs

We are developing a number of disease-modifying gene therapy candidates to treat larger-rare cardiovascular diseases that have significant unmet need and no approved treatments that address the underlying genetic cause of the disease. These programs include:

- *LX2006* is an AAVrh10-based gene therapy candidate designed to intravenously deliver a functional frataxin, or *FXN*, gene for the treatment of FA cardiomyopathy. FA cardiomyopathy is the most common cause of mortality in patients with FA and affects approximately 5,000 patients in the United States. *LX2006* is designed to promote the expression of the protein frataxin to restore normal mitochondrial function and energy production in myocardial cells. In preclinical studies, *LX2006* demonstrated improvement in cardiac function and survival in a severe *FXN* knockout mouse model. Similarly, in a partial *FXN* knockout mouse model, *LX2006* was observed to restore cardiac function and reverse disease abnormalities of FA cardiomyopathy. *LX2006* is currently being evaluated in an open-label, ascending dose Phase 1/2 clinical trial in patients with FA cardiomyopathy and we have observed an increase in frataxin protein expression in the hearts of three patients that have undergone cardiac biopsies across cohort 1 (n=1) and cohort 2 (n=2). We expect to report additional interim data from this trial in mid-2024. The FDA has granted Rare Pediatric Disease designation and Orphan Drug designation to *LX2006* for the treatment of FA.
- *LX2020* is an AAVrh10-based gene therapy candidate designed to intravenously deliver a functional *PKP2* gene to cardiac muscle for the treatment of PKP2-ACM. *PKP2* mutations are associated with approximately 75% of all genetic cases of ACM, and we estimate they affect approximately 60,000 patients in the United States. *PKP2* mutations can cause replacement of heart muscle with fibrotic

tissue and fatty deposits and severe abnormal heart rhythms, or arrhythmias, that cause cardiac dysfunction and can result in sudden cardiac death. LX2020 is designed to increase desmosomal PKP2 protein levels, reassemble desmosomes and restore myocardial cell function. In our preclinical studies using a genetic mouse model of ACM harboring a *PKP2* mutation that recapitulates the phenotype of PKP2-ACM, LX2020 resulted in fewer arrhythmias and increased survival. We received IND clearance for LX2020 from the FDA in July 2023 and Fast Track and Orphan Drug designations from the FDA in December 2023. We expect to dose the first patient in a Phase 1/2 clinical trial in the first half of 2024 and provide an interim data readout from cohort 1 in the second half of 2024.

- *LX2021* is a gene therapy candidate we are developing to intravenously deliver the coding sequence for the functional Cx43 protein for a group of inherited cardiac muscle disorders associated with a high risk of sudden death, including ACM and certain forms of dilated cardiomyopathy. We believe restoring the Cx43 protein can potentially treat multiple genetic causes of ACM because the cardiac loss of Cx43 is a molecular deficit generally observed in all ACM patient populations. Our LX2021 program is initially targeting Desmoplakin, or DSP, cardiomyopathy, a distinct form of ACM as well as a certain form of dilated cardiomyopathy, with a prevalence that may be as high as 4% of all inherited dilated cardiomyopathies and 4% of all ACMs, impacting up to approximately 35,000 patients in the United States. We plan to initiate IND-enabling studies for LX2021 in 2024.
- *LX2022* is a gene therapy candidate we are developing to intravenously deliver a functional *TNNI3* gene to myocardial cells to treat a distinct form of hypertrophic cardiomyopathy, or HCM, due to mutations in the *TNNI3* gene. Mutations in the *TNNI3* gene often result in left ventricular hypertrophy and restrictive cardiomyopathy, leading to arrhythmias and heart failure. With an estimated prevalence of 1 in 500 people in the United States, HCM is one of the most common forms of genetic cardiomyopathy caused by mutations that affect the cardiac sarcomere in approximately 75% of cases. It is estimated that as many as 25,000 patients in the United States and 34,000 patients in the European Union are affected by HCM caused by mutations in the *TNNI3* gene. We plan to complete candidate selection for LX2022 in 2024.

APOE4-associated Alzheimer's programs

We are developing a portfolio of approaches to treat the genetics underlying Alzheimer's disease. These programs include:

- *LX1001* is an AAVrh10-based gene therapy candidate designed to deliver into the cerebrospinal fluid, or CSF, an *APOE2* gene for the treatment of *APOE4* homozygous patients with Alzheimer's disease. Alzheimer's disease is the leading cause of cognitive decline in late adult life and characterized by complex underlying pathology in the central nervous system, or CNS. Individuals homozygous for *APOE4*, an allele of the gene *APOE*, are approximately 15 times more likely to develop Alzheimer's disease than the general population, and it is estimated that there are 900,000 *APOE4* homozygous patients with Alzheimer's disease in the United States alone. Conversely, individuals homozygous for the *APOE* allele *APOE2* are 40% less likely to develop Alzheimer's disease than the general population. This and other evidence suggest that *APOE2* may play a neuroprotective role. LX1001 is designed to express the protective *APOE2* gene in the CNS of *APOE4* homozygous patients in order to halt or slow the progression of Alzheimer's disease. LX1001 is being evaluated in an ongoing open-label, dose-escalation Phase 1/2 clinical trial. In the first dose cohort in the trial, we observed a consistent trend towards improvement in Alzheimer's disease CSF biomarkers, such as total tau and phosphorylated tau. We have also observed expression of the protective *APOE2* protein in all patients in the first dose cohort with follow-up data. LX1001 has been granted Fast Track designation by the FDA for the treatment of patients with early Alzheimer's disease who are *APOE4* homozygous to slow disease progression. We completed enrollment in the trial in the fourth quarter of 2023 and expect to report additional interim data from all cohorts in the Phase 1/2 clinical trial in the second half of 2024.
- *LX1021* is a gene therapy candidate we are developing to deliver a Christchurch mutation-modified *APOE2* allele for the treatment of *APOE4* homozygous patients with Alzheimer's disease. The

Christchurch mutation has been recognized to protect individuals against Alzheimer's disease even in the presence of significant amyloid pathology. The mechanism of this protection may relate to the fact that APOE, in the presence of the Christchurch mutation, binds poorly to heparan sulfate proteoglycans, which are molecules found on the surface of neurons that may inhibit the spread of tau between cells. We believe this approach has the potential to enhance the protective effect of APOE2 in homozygous *APOE4*-associated Alzheimer's disease. We plan to hold a pre-IND meeting with the FDA in 2024 to guide the next stage of development for this program.

- *LX1020* is a gene therapy candidate we are developing to deliver both the protective *APOE2* allele and microRNA, or miRNA, to suppress *APOE4* for the treatment of *APOE4* homozygous patients. We believe delivery of *APOE2* with concurrent suppression of *APOE4* will achieve a higher degree of conversion to the *APOE4/E2* heterozygous profile, which should lead to greater therapeutic effect. We plan to complete candidate selection in 2024.

Our company

We are led by pioneers and experts with decades of collective experience in genetic medicines, rare disease drug development, manufacturing and commercialization. We were founded through a collaboration between the world-class gene therapy research at Joan & Sanford I. Weill Medical College, or Weill Cornell Medicine, and a team of pioneering scientists, clinicians, and business leaders with deep expertise in gene therapy. Through our acquisition of Stelios Therapeutics, Inc., or Stelios, we have an ongoing collaboration with the University of California, San Diego, or UCSD, through which we sponsor research and development designed to advance our earlier stage cardiovascular pipeline. We work with highly experienced teams at both Weill Cornell Medicine and UCSD that have deep expertise in the underlying biology of cardiovascular and *APOE4*-associated Alzheimer's disease and the patient populations that they treat. We believe our ongoing work with these preeminent institutions, as well as any potential new or expanded collaborations, will continue to be a valuable aspect of our efforts as we seek to further discover and develop novel gene therapies for devastating diseases.

Our strategy

Our company is purpose built to amplify genetic medicine's potential for empowering individuals by treating the underlying cause of genetic disease. The key elements of our strategy to achieve this vision are to:

- *Focus our AAV-based gene therapy candidates in areas of high unmet need and with substantial potential for societal impact and commercial opportunity, such as genetically defined cardiovascular diseases and *APOE4*-associated Alzheimer's disease.* We are focused on genetically defined cardiovascular diseases and *APOE4*-associated Alzheimer's disease where there are no currently approved treatments or where we believe our therapies will provide a meaningful improvement relative to existing standards of care. Furthermore, we pursue indications with commercial opportunities beyond those typically associated with gene therapy companies targeting rare monogenic diseases. Among our cardiovascular programs, LX2006 has the potential to address a prevalent population of approximately 5,000 FA cardiomyopathy patients in the United States, and LX2020 has the potential to address approximately 60,000 patients in the United States with PKP2-ACM. We believe that LX1001 has the potential to address up to approximately 900,000 patients in the United States with homozygous *APOE4*-associated Alzheimer's disease.
- *Advance a deep and diverse pipeline that includes candidates that are designed to address both larger-rare and more prevalent patient populations, prioritizing conditions most likely to benefit from our therapies.* Our programs are designed to target either the aspect of the disease that is most amenable to gene therapy or the genetic sub-groups that we believe are most likely to respond to gene therapy. For example, in the case of LX2006, we are targeting the cardiac manifestation of the disease because we believe it is more amenable to gene therapy than the neurologic manifestation. For LX2020, we are targeting the PKP2 sub-group that represents approximately 75% of the genetic ACM cases and

presents a clinical phenotype amenable to treatment. For LX1001, we are targeting homozygous APOE4-associated Alzheimer's disease patients, the highest risk population to develop Alzheimer's disease and the sub-group most likely to demonstrate a treatment effect from the expression of the protective APOE2 protein and where currently approved treatments have demonstrated clinical benefit less favorable compared to other APOE genotypes.

- *Pursue a staged, capital-efficient approach for advancing programs through clinical development and regulatory approval.* We are advancing our pipeline of AAV-based gene therapies for cardiovascular and APOE4-associated Alzheimer's disease. We expect to report additional data from our Phase 1/2 clinical trial of LX2006 for the treatment of FA cardiomyopathy in mid-2024. We completed enrollment in the ongoing Phase 1/2 trial of LX1001 for the treatment of APOE4-associated Alzheimer's disease in the fourth quarter of 2023, and we expect to report additional interim data from all cohorts in the second half of 2024. We plan to leverage key learnings from our lead programs in cardiovascular and APOE4-associated Alzheimer's disease to guide the development of our next-generation candidates and follow-on indications. Given that there are several targets in cardiac organelles that are dysregulated in cardiomyopathy, we believe there will be read-through to similar indications following clinical proof-of-concept from our initial FA cardiomyopathy and PKP2-ACM programs. Similarly, we believe that positive clinical data from our APOE4-associated Alzheimer's disease program has the potential to validate our novel approach to treating the APOE4 homozygous sub-group and can help inform the development plan of our next-generation candidates. Furthermore, we plan to leverage biomarkers for each of our development programs, potentially enabling us to rapidly validate proof-of-mechanism and support further development efforts. We believe our strategy could facilitate shorter clinical trial timelines, which, coupled with expedited global regulatory approval pathways we plan to pursue, could potentially accelerate clinical development and reduce overall development costs.
- *Utilize a unified, high-quality manufacturing platform that can quickly respond at scale to high impact opportunities.* We plan to use a unified manufacturing process across all current and future programs to manufacture vector for clinical and commercial use via a proprietary platform suspension baculovirus process using Sf9 cells. This production platform has demonstrated the capability to produce high-quality, high-yield, and high-potency vectors that can accommodate demand for both larger rare and prevalent patient populations. We are currently working with third parties for our manufacturing capabilities; however, we will evaluate building out our own current good manufacturing practices, or cGMP, - compliant manufacturing facility as, and if, we achieve further clinical success or any of our product candidates ultimately receive marketing authorization.
- *Pursue next-generation genetic medicine technologies that can enhance our capabilities and expand our impact on patients.* We have combined our robust expertise in vector design, regulatory and clinical strategy, and disease area knowledge to establish a deep cardiovascular gene therapy pipeline. We have chosen to use AAVrh10 across our initial programs and will continue to evaluate the latest scientific understanding of capsid technology for each of our future programs. We will continue to pursue the development of novel capsids, promoter technologies, routes of delivery and other next-generation capabilities to optimize the potential therapeutic efficacy of our programs through our own efforts and in collaboration with others. In August 2023, we announced a strategic investment from Sarepta Therapeutics, Inc. to explore collaboration opportunities within our preclinical cardiovascular pipeline. We may explore additional opportunities to forge strategic partnerships and collaborations in the future.
- *Leverage and expand upon our partnerships and exclusive licenses with world-class academic institutions.* Our foundational science stems from partnerships and exclusive licenses with leading academic laboratories at Weill Cornell Medicine and UCSD, two preeminent institutions on the cutting edge of gene therapy research. Of note, our Chief Scientific Advisor, Ronald G. Crystal, M.D., has sponsored 14 cleared gene therapy IND applications across multiple disease areas, and researchers at UCSD led the discovery efforts of a cardiovascular gene therapy program that is expected to move into

late-stage clinical trials. We will continue to draw on the scientific expertise provided by these partnerships while evaluating new opportunities for complementary research and development with other academic collaborators.

- *Build a fully integrated genetic medicine company and selectively evaluate strategic opportunities to maximize the impact of our pipeline.* We aim to discover, develop, manufacture, and eventually commercialize our gene therapy candidates. Despite the larger populations in most of our target indications, we believe genetically defined patient populations enable us to pursue a rare disease commercialization strategy characterized by targeted patient identification and facilitating an efficient patient journey through the diagnostic process, engagement with patient advocacy groups, the potential for value-based market access efforts, and sales, account management, and field reimbursement teams focused on the small number of centers that typically diagnose and treat genetic diseases. We may seek strategic collaborations where we believe the resources and expertise of third-party pharmaceutical or biotechnology companies could accelerate the clinical development or maximize the market potential of our product candidates, or where such collaborations could expand our internal capabilities and platform technology. In particular, our LX1001 program may benefit from a co-development partnership to help us take the program through later stages of development.

Our approach

Background and successes of gene therapy

Gene therapy is one of the most important emerging modalities, given its potential, in a single administration, to treat or cure life threatening diseases. Other non-genetic medicine approaches have alleviated certain symptoms and conditions associated with genetic diseases, but they do not directly address the underlying genetic cause of the disease.

Gene therapies are designed to deliver transgenes, which are functional versions of the genes that are mutated or are the cause of deficient proteins that manifest in genetic diseases. These transgenes are utilized by the body's cellular machinery to naturally produce functional proteins that were deficient or non-functional prior to treatment. The production of these functional proteins is intended to provide consistent and durable therapeutic benefit. Gene therapies are typically comprised of three key components: a vector—a vehicle that delivers a transgene to cells in the body; a transgene—a gene intended to produce a functional protein; and a promoter—a specialized DNA sequence that directs cells to initiate transcription. AAV-based gene therapy has been shown to be highly effective in targeting many organ systems and has been studied in more than 3,300 patients worldwide.

Although gene therapies have been studied in human clinical trials for over 30 years, there are relatively few AAV-based gene therapy products approved by the FDA. However, less than four years after its 2019 FDA approval, voretigene neparvovec rzyl (Luxturna), a treatment of a rare ocular condition, generated \$1.4 billion of net product sales in 2022, highlighting the commercial viability of novel gene therapies.

Our gene therapy approach

Our integrated modular approach enables us to optimize our strategy to pursue larger-rare and prevalent genetically defined indications in specific sub-groups of patients. Our gene therapy candidates utilize the vector construct, dose and route of administration that we believe will result in the most favorable biodistribution profile for each disease. By specifically tailoring our technological approach to each targeted disease, we believe we can optimize treatments to achieve the highest likelihood of having potential therapeutic impact. Our disease area strategy is focused on defined sub-groups within selected cardiovascular diseases and APOE4-associated Alzheimer's disease that we believe are most amenable to gene therapy. Across our portfolio, we are utilizing a scalable baculovirus/Sf9 expression system that has demonstrated the capability to produce high-quality, high-yield, and high-potency vectors that can accommodate demand for both rare and prevalent patient populations. Finally, we continuously seek to bolster our pipeline through relationships with academic institutions, providing

us with access to cutting edge genetic medicines research which will include not only AAV gene therapy but also other potential therapeutics payload types and non-viral delivery systems.

Our technology approach

Our most advanced cardiac and APOE4-associated Alzheimer's disease programs use the AAVrh10 vector due to its high transduction efficiency in both myocardial cells and neurons, higher ratio of cardiac to liver biodistribution, potentially lower toxicity given the ability to utilize lower doses, compared to other well-established AAV serotypes, and lower pre-existing immunity. We believe that our approach to technology has the potential to confer the following advantages over existing gene therapy technologies:

High Transduction Efficiency and Biodistribution. The AAVrh10 vector has been shown to be capable of transducing myocardial cells based on preclinical research, and it has been shown to be capable of transducing neurons based on results from preclinical studies and our ongoing clinical trials. We believe these results demonstrate that among currently available, commonly used serotypes, AAVrh10 is an efficient vector for delivery and expression of transgenes for the treatment of the cardiovascular diseases that we are currently targeting and APOE4-associated Alzheimer's disease. We have observed organ-specific biodistribution advantages for our AAVrh10 vector compared to AAV9, a well-known serotype that has been evaluated in several clinical trials, including one cardiovascular clinical trial. For example, we have observed vector distribution of AAVrh10 in cardiac tissue of non-human primates, or NHPs, that is two times greater than for AAV9. Additionally, our preliminary studies in Yucatan minipigs have shown that AAVrh10 has approximately 1.5 times greater biodistribution in the heart than AAV9. In addition, preclinical studies conducted by Weill Cornell Medicine have demonstrated that systemic administration of AAVrh10 promotes a ratio of cardiac-to-liver biodistribution that is more favorable than what is known of other commonly used vector serotypes. Preclinical data from a study using a murine model conducted by other researchers has demonstrated that more AAVrh10 particles transduced a single brain cell than AAV9. Additionally, third party researchers observed higher transduction efficiency of AAVrh10 compared to AAV9 in all brain regions evaluated in a preclinical murine study.

Reduced Toxicity. The cardiac tropism of AAVrh10 may allow our gene therapy candidates to be systemically administered at lower doses than many other AAV-based therapies targeting cardiovascular or other systemic diseases. We have selected target indications that we believe will be responsive to AAVrh10-based therapies administered at comparatively lower doses, which will potentially reduce toxicities that have been reported in clinical trials of higher-dose gene therapies. For example, compared to doses used in other systemic gene therapy programs which can exceed 1×10^{14} genome copies per kilogram, we intend to use doses of low $\times 10^{11}$ to low $\times 10^{12}$ genome copies per kilogram in our Phase 1/2 clinical trial of LX2006 for the treatment of FA cardiomyopathy. Similarly, early data suggest a lower dorsal root ganglia toxicity profile than other commonly used vectors, making it amenable for treating APOE4-associated Alzheimer's disease at potentially higher doses than other vectors.

Reduced Pre-Existing Neutralizing Antibodies. Treatments leveraging vector serotypes to which humans have pre-existing immunity tend to be less effective. Among the naturally occurring and commonly used AAV serotypes, AAVrh10 has been shown in preclinical studies to have among the lowest levels of pre-existing neutralizing antibodies.

Optimized Expression. We are collaborating with our academic partners to develop tissue-specific promoters and enhancers, tissue-specific codon optimization and tissue-specific, miRNA-based knockdown which can be utilized to limit transgene expression in non-target organs. We are also exploring additional genetic medicine approaches to increase tissue tropism using novel capsids and developing next generation expression systems to optimize the potential therapeutic efficacy of our product candidates.

Our precision medicine focus

We are focused on disease areas that have seen limited penetration of precision medicines. The development of precision medicines has transformed treatment paradigms in certain therapeutic areas. For example, the top five precision medicines approved in oncology between 2016 and 2021 have a combined estimated U.S. annual peak sales of approximately \$25 billion. Conversely, relatively few precision medicines have been approved for treating cardiac or CNS conditions; this is despite a rapid advancement in the understanding of the genetic underpinning of cardiovascular and neurologic diseases and the improved ability of viral vectors to transduce the heart and brain safely.

Cardiovascular disease remains the most significant cause of morbidity and mortality in the western world and is rapidly becoming a primary cause of death worldwide. We believe the current lack of precision medicines across the cardiovascular landscape is due to historical regulatory precedents focused on “hard endpoints” and delivery technology that has been insufficiently cardiotropic. However, recent approvals by the FDA suggest a willingness to approve new therapies based on biomarkers and functional endpoints. For example, a competitor recently announced FDA alignment on the design of its pivotal study for RP-A501 for the treatment of Danon disease. The 12 patient study will utilize a co-primary endpoint consisting of protein expression and a reduction in left ventricular mass, with troponin noted as a key secondary endpoint, and it will have an external comparator arm. We believe this highlights the potential to utilize similar biomarkers for other genetic cardiomyopathies with high unmet need, subject to regulatory approval.

Furthermore, modern AAV delivery technology generally targets the heart and mediates the delivery of genetic payloads. These developments should foster the introduction of genetic medicines in the cardiovascular treatment landscape, offering one of the most substantial opportunities for the uptake of precision medicines in the global pharmaceutical marketplace. There is significant potential for cardiovascular precision medicine. For example, approximately 30% of cases of dilated cardiomyopathy, a common cause of heart failure, stem from genetic mutations in which more than 50 distinct genes have been implicated. Advancements in genome sequencing have led to increasing identification of these genetic mutations over the last two decades. In the majority of genetic cardiomyopathies, there is no existing disease-modifying therapeutic option. In cases where a disease-modifying treatment option is available, the current standard of care only forestalls disease progression. We believe our cardiovascular precision medicine focus offers the opportunity to treat the underlying root cause of the disease and more effectively reverse disease progression with a single dose.

Precision medicine has been more relevant across CNS disorders, in particular gene therapies for ophthalmologic and neuromuscular degeneration. However, there has been limited advancement in developing precision medicines for Alzheimer’s disease, the most significant commercial opportunity within the CNS landscape. *APOE* is the leading genetic risk factor for Alzheimer’s disease, and, despite differences in treatment effect and safety seen between subpopulations in recent clinical trials, currently-approved, disease-modifying treatments for Alzheimer’s disease utilize a “one-size-fits-all” approach, regardless of *APOE* subpopulation. We believe this highlights the unmet need for precision medicines as the Alzheimer’s disease treatment paradigm continues to shift.

We believe a precision medicine focus for cardiovascular diseases and APOE4-associated Alzheimer’s disease represents a compelling opportunity and the next frontier in expanding the impact of genetic medicines across therapeutic areas:

- *Precision genetic medicines target the underlying cause of the disease:* next-generation, precision-based genetic medicines offer the opportunity to directly target the mechanisms underlying cardiovascular disease and Alzheimer’s disease.
- *Significant unmet need in cardiovascular disease and APOE4-associated Alzheimer’s disease:* more than 30 million adults have been diagnosed and approximately 655,000 Americans die yearly from heart disease. Additionally, we believe there are approximately 900,000 *APOE4* homozygous patients

in the United States, and current treatment options are likely to be less effective for this subpopulation of patients.

- *Improved landscape of regulatory precedents utilizing cardiac and CNS biomarkers:* biomarkers can help accelerate development timelines, reduce patient attrition, and reduce the overall cost of drug development by providing an early read on potential efficacy and therapeutic impact.
- *Improved diagnostics expand market opportunity:* implementation of genetic testing has the potential to help identify patients, including those who are asymptomatic or at a higher likelihood of developing the disease being evaluated or who respond less favorably to currently approved treatments.

Our disease area strategy

Our cardiovascular and APOE4-associated gene therapy programs are designed to have the following characteristics:

- *Indications that may be effectively treated by gene therapy.* We select targets that correspond to populations with a specific genetic profile and clearly defined disease phenotype.
- *Indications with the potential to demonstrate early evidence of meaningful clinical benefit.* We pursue clearly defined biomarkers and functional endpoints that can provide early proof-of-mechanism and inform clinical development decisions, including the potential to seek accelerated approval pathways. Early data regarding functional endpoints and biomarkers allows us to pursue an efficient capital allocation strategy. Certain of our clinical trials are designed to provide initial biomarker data as early as three months from the treatment date. In the case of LX2006 targeting FA cardiomyopathy and LX2020 for PKP2-ACM, we are also pursuing functional and histological endpoints that may demonstrate signs of meaningful clinical benefit as early as three months. We expect these early signals will enable us to design late-stage clinical studies with endpoints that best mirror therapeutic effects that may cure the disease. In addition, we believe these endpoints should allow us to perform smaller trials that can be completed in a more reasonable time frame than traditional cardiovascular trials.
- *Present opportunity to address high unmet medical need.* We are focused on genetically defined cardiovascular diseases and APOE4-associated Alzheimer's disease where there are no currently approved treatments or where we believe our therapeutic candidates will have a meaningful improvement relative to existing standards of care.
- *Significant market opportunity.* We pursue indications with significant commercial opportunities beyond those typically associated with gene therapy companies targeting rare monogenic diseases. Our current focus is on targets that impact larger-rare disease populations, ranging from approximately 5,000 U.S. patients, in the case of FA cardiomyopathy, to approximately 60,000 U.S patients, in the case of PKP2-ACM, or prevalent diseases such as homozygous APOE4-associated Alzheimer's disease, which has a patient population of up to approximately 900,000 in the United States.
- *Targets that have established proof-of-concept.* We have leveraged our relationships with academic institutions including Weill Cornell Medicine and UCSD to in-license product candidates with established proof-of-concept in relevant preclinical models that closely resemble the clinical phenotype we are pursuing. We will continue to seek additional similar opportunities via collaborations with academia and the industry.
- *Targeted disease areas best treated by optimal delivery technologies.* Our current gene therapy candidates utilize the AAVrh10 vector due to its tropism for the heart and its observed tolerability in clinical trials for CNS disorders. We will pursue the optimal technology to address the diseases of interest for future indications while ensuring sufficient preclinical or clinical validation for any novel approaches.
- *Readily accessible patients.* Our goal is to accelerate patient recruitment for our clinical trials and increase the likelihood of commercial success of our potential products by focusing on diseases with

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established patient advocacy groups and university researchers who maintain registries of potentially eligible patients. Where possible, we leverage existing natural history studies to better define target patient phenotypes associated with the disease and may utilize these natural history datasets as a control group in future studies.

Our manufacturing approach

We are developing gene therapy candidates for larger-rare and prevalent disease patient populations that require a high-quality process that can produce vectors in relatively large quantities while utilizing traditional biologics manufacturing infrastructure. We utilize a baculovirus/Sf9 expression system to manufacture our gene therapy candidates. Our manufacturing platform is designed to infect Sf9 cells at high densities in suspension cell culture with both an AAVrh10 and baculovirus containing the transgene. The output is coupled with a chromatography-based purification process which allows for efficient AAV purification, resulting in higher yields and fewer empty AAV capsids than traditional plasmid HEK adherent cell culture approaches.

Traditional adherent HEK manufacturing approaches, which use plasmid transfection to produce viral vectors, are based on mammalian-derived cell lines and cellular components that replicate in mammalian cells. We believe our process has an improved safety profile over these manufacturing approaches for the following reasons: through our sponsored next-generation PacBio and Illumina sequencing analyses performed by one of our CROs, we have observed reduced incorporation of non-transgene DNA plasmid DNA impurities, from 15% observed in certain adherent HEK systems to 0.2% in our process; our cellular components are non-replicating in mammalian cells; we have eliminated potentially immunogenic or toxic animal-derived proteins; and our cells are grown under serum-free conditions, leading to reduced risk of contamination from animal-derived products.

We believe our manufacturing process enables us to efficiently pursue our goal of targeting larger-rare and prevalent patient populations. Historically, manufacturing challenges, largely driven by the quantity of vector required to pursue large commercial opportunities have limited the utility of gene therapy for large patient populations. We believe our proprietary SF9 baculovirus process will allow us to produce vectors at the necessary scale to support the patient populations we are targeting at a cost-of-goods profile similar to what the pharmaceutical marketplace has seen with biologics.

First generation SF9 baculovirus platforms have historically been less infectious than traditional HEK systems, which is associated with lower levels of the capsid viral protein 1, or VP1, one of the three viral proteins that make up the AAV capsid. However, we have implemented the Virovek “Bac to AAV” technology which enables wild type levels of VP1 allowing us to achieve potency levels largely in-line with a HEK approach. This has been observed in an *in-vitro* potency assessment of two lots of LX2006 comparing HEK and SF9 manufacturing approaches which resulted in an equivalent potency between manufacturing methods.

As a representative example of our process, we achieved purified yield levels of greater than 1×10^{14} vector genomes per liter of cell culture in a 50-liter run manufactured for our LX2020 IND-enabling NHP safety studies with full capsid levels of approximately 70%. In a triple transfection HEK suspension manufacturing process for AAVrh10 produced by a third party contract manufacturing organization, or CMO, publicly available data demonstrated purified yields of approximately 1×10^{13} vector genomes per liter of cell culture, which we believe is a reasonable estimate of industry norms. Our manufacturing results are approximately 10 times more yield efficient than these levels. We believe this demonstrates the feasibility of our process and our ability to provide late-stage clinical and commercial material in a cost-efficient manner.

Based on our approach, we expect that our existing partnerships can supply material for all of our currently ongoing and planned clinical trials as well as potential commercial production of some of our programs. We have secured relationships with Virovek, Inc. for its “Bac-to-AAV” system, Millipore Corporation for its RVN Sf9 cell line, and Fujifilm Diosynth Biotechnologies U.S.A., Inc. for viral vector process development and cGMP manufacture of viral vectors. We will own the intellectual property created by our manufacturing process

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development activities or have the ability to license it and will maintain the option to transfer the process to other CDMOs in the future and/or to our own potential facility to ensure ongoing redundancy and reliability.

Academic collaborations

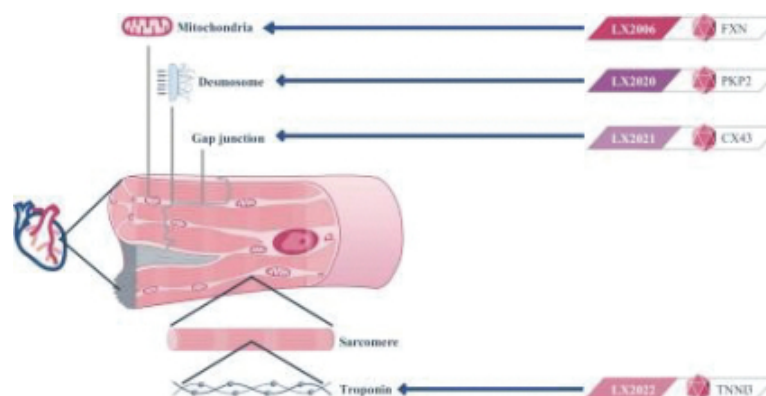
To support our integrated modular approach, we have partnered with leading academic institutions who are on the cutting edge of gene therapy research. Our relationships with Weill Cornell Medicine and UCSD have provided us with access to the latest gene therapy research. Our collaboration efforts with Weill Cornell Medicine have focused on the discovery of second- and third-generation cardiac vector technology and novel cardiac transcriptional promoters, all of which have the potential to be deployed in our early-stage research efforts. Our collaboration with UCSD focuses on preclinical candidate selection and translational research in several larger-rare cardiovascular disease indications. Both institutions are recognized leaders in gene therapy, and the collaborations are designed to best leverage their respective skill sets. We may seek additional academic collaborations where such collaborations could expand our internal capabilities and platform technology.

Our cardiovascular gene therapy programs

We have combined our robust expertise in cardiac-targeted vector design, regulatory and clinical strategy, and disease area knowledge to establish a deep cardiovascular gene therapy pipeline. We are developing a number of disease-modifying gene therapy candidates to treat larger-rare cardiovascular diseases that have significant unmet need and no approved disease-modifying treatments. Our most advanced program, LX2006, is an AAVrh10-based gene therapy candidate for the treatment of FA cardiomyopathy caused by mutations in the *FXN* gene. We are also advancing several other AAV-based gene therapy programs to treat additional genetically defined cardiac diseases, including LX2020 to treat ACM caused by mutations in the *PKP2* gene, LX2021 to treat DSP cardiomyopathy associated with Cx43 deficiency, and LX2022 to treat HCM associated with mutations in the *TNNI3* gene. We plan to leverage our experience and learnings from LX2006 and LX2020 and apply them to our earlier-stage cardiac programs.

As shown in the graphic below, our cardiovascular genetic medicine strategy is focused on initial candidates that target a range of critical organelles that are dysregulated in various genetic cardiomyopathies, including the mitochondria, the desmosome, gap junctions, and the sarcomere. We believe that the learnings from our initial development efforts will provide the basis for us to pursue additional discovery efforts targeting other cardiac indications implicated in these tissues.

Several targets in cardiac organelles are dysregulated in various cardiomyopathies



LX2006 for the treatment of FA cardiomyopathy

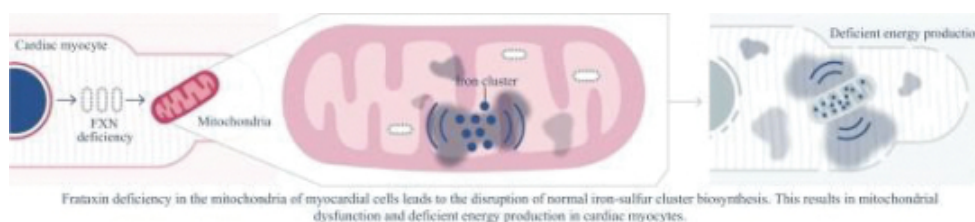
LX2006, an AAVrh10-based gene therapy candidate for the treatment of FA cardiomyopathy, is designed to deliver functional *FXN* intravenously to promote the expression of frataxin in order to restore normal mitochondrial function in myocardial cells. Cardiomyopathy is the most common cause of mortality in patients with FA. The FDA has granted Rare Pediatric Disease designation and Orphan Drug designation to LX2006 for the treatment of FA cardiomyopathy. LX2006 is currently being evaluated in an open-label, dose-escalating Phase 1/2 clinical trial in patients with FA cardiomyopathy and we have observed an increase in frataxin protein expression in the hearts of three patients that have undergone cardiac biopsies across cohort 1 (n=1) and cohort 2 (n=2). We expect to report additional interim data from this trial in mid-2024.

Overview of FA

FA is a genetic, progressive, and degenerative multi-system disorder with a prevalence of 1:50,000 or approximately 6,600 people in the United States. It is estimated that approximately 80%, or 5,000 of these patients, will develop FA cardiomyopathy. In the European Union, FA affects approximately 9,000 people, with approximately 7,000 of these cases projected to include FA cardiomyopathy. FA is caused by a mutation in the *FXN* gene that disrupts the normal production of the protein frataxin, which is critical to the function of mitochondria in a cell and to the maintenance of cardiac function. The most common FA phenotype presents with significantly reduced frataxin levels compared to healthy individuals. *In vivo* research evaluating frataxin protein levels in non-cardiac tissues, such as buccal cells, peripheral blood mononuclear cells and whole blood, demonstrates that FA heterozygote carriers, who present with no disease symptoms, exhibit approximately 2.5-fold greater frataxin levels when compared to FA patients. The levels of frataxin in the cardiac tissue of heterozygote carriers compared to FA patients is unknown.

Third-party researchers recently developed a new FA murine model with approximately 800 guanine-adenine-adenine repeats, the YG8-800 model, which has approximately 5% of normal frataxin levels in the heart and yet displays near normal cardiac function. We believe this model, along with findings from our own preclinical studies, demonstrate that relatively low levels of frataxin in the heart may be sufficient to maintain cardiac function. The frataxin deficiency in the mitochondria of myocardial cells in FA patients causes disruption of normal iron-sulfur cluster biosynthesis leading to mitochondrial dysfunction and deficient energy production, as shown below.

Overview of FA cardiomyopathy disease mechanism



The neurologic disease and cardiac disease are two distinct manifestations of FA. The disease is inherited in an autosomal recessive manner, where both inherited genes are abnormal, and symptoms usually begin in childhood. Absence of fully functional frataxin leads to damage to peripheral nerves and the parts of the brain that controls movement and balance, leading to neurological symptoms that include impaired muscle coordination, or ataxia, that worsen over time. Initial symptoms may include unsteady posture, frequent falling, and progressive difficulty in walking due to impaired ability to coordinate voluntary movements. Affected individuals often develop slurred speech, hearing loss, scoliosis, diabetes, characteristic foot deformities, and an irregular curvature of the spine. The typical age of onset of neurological symptoms is five to 15 years old. As the disease progresses, patients typically experience various heart conditions, including thickening of the heart

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muscle, or hypertrophic cardiomyopathy, and arrhythmias. Hypertrophic cardiomyopathy, fibrosis, heart failure and arrhythmias are the cause of death in approximately two-thirds of FA patients. Typical onset of the cardiac disease is at 15 to 30 years old.

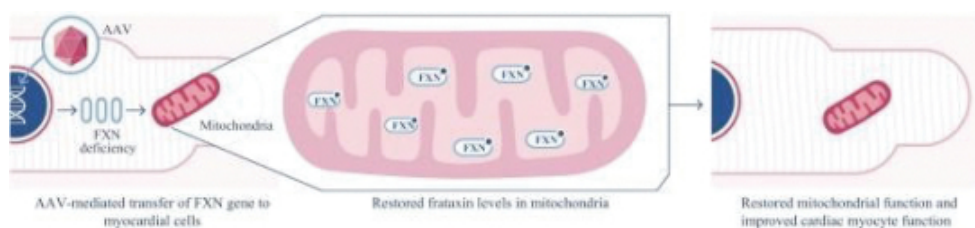
By the time the cardiac disease of FA emerges, the neurologic disease is generally significantly advanced and may not be amenable to gene therapy. There are currently no approved treatments for the cardiac manifestations of FA. *Skyclarys*, the only approved treatment for FA, is an Nrf2 activator which only targets the CNS component of the disease. As a result, patients with FA have significant unmet need for a therapy to treat cardiovascular complications associated with the disease.

Our approach: LX2006

We are developing LX2006 as an AAVrh10-based gene therapy delivered intravenously for the treatment of FA cardiomyopathy. LX2006 is designed to deliver the *FXN* gene under the transcriptional control of the CAG promoter, a strong synthetic promoter frequently used in viral vectors. LX2006 utilizes AAVrh10 based on its favorable cardiac affinity and vector distribution profile observed in preclinical studies, as compared to AAV9. In preclinical studies, vector distribution in cardiac tissue with AAVrh10 has been observed to be between 1.5 to 2 times the distribution associated with the use of AAV9.

LX2006 is designed to transfer the *FXN* gene to myocardial cells and increase frataxin levels in the mitochondria. The increase in frataxin levels in the mitochondria is intended to restore mitochondrial function and energy production in cardiac myocytes, as shown below.

LX2006 is designed to deliver functional frataxin in order to restore normal mitochondrial function



Lexeo-sponsored LX2006 clinical development and trial design

LX2006 is currently being evaluated in an ongoing Phase 1/2 clinical trial (SUNRISE-FA) in patients with FA cardiomyopathy. SUNRISE-FA is a first-in-human, 52-week, dose-ascending, open-label trial of LX2006 in patients who have FA cardiomyopathy. LX2006 is administered as a one-time intravenous infusion with prednisone utilized for immune suppression. The trial consists of at least three patients in each cohort, with the potential for cohort expansion. The cohort 1 dose level is 1.8×10^{11} vg/kg, the cohort 2 dose level is 5.6×10^{11} vg/kg, and the cohort 3 dose level is 1.2×10^{12} vg/kg. There will be a long-term follow-up for patients who receive LX2006 to monitor ongoing safety for a total of five years, per FDA requirement.

Key patient inclusion criteria for the study include genotyping, left ventricular hypertrophy and echocardiography, or ECHO, changes, and ejection fraction $\geq 40\%$. The primary endpoint of the Phase 1/2 trial is to assess the safety and efficacy of one-time administration of LX2006 for the purpose of selecting the appropriate dose for further clinical development. Exploratory endpoints assess biomarkers, including cardiac frataxin expression levels, and preliminary functional efficacy assessments relevant in evaluating the ability of LX2006 to stop progression and improve FA cardiomyopathy. The following biomarker, functional and imaging efficacy assessments will be conducted:

CPET: Peak VO₂ and other functional measures will be performed at regular intervals.

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Cardiac MRI: All patients undergo MRI scans of the heart to measure cardiac hypertrophy, left ventricular ejection fraction, stroke volume, strain, and fibrosis.

Cardiac ECHO: All patients undergo cardiac ECHO to measure cardiac hypertrophy, left ventricular ejection fraction and strain.

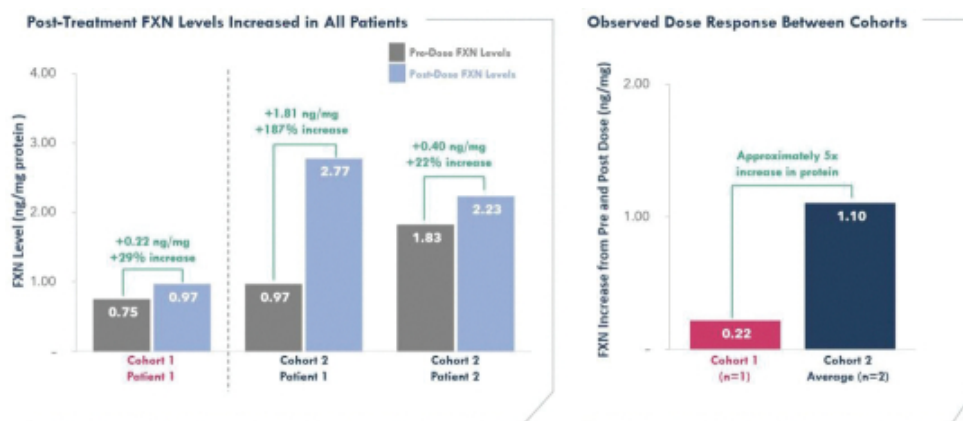
Cardiac biopsy: Biopsies will measure, among other things, frataxin protein expression.

Biomarkers: Serum cardiac biomarkers, including troponin.

Cardiac Biopsy Results from Cohort 1 and Cohort 2 of SUNRISE-FA Trial

To precisely evaluate frataxin levels in human cardiac tissue samples, we utilized a novel research grade liquid chromatography mass spectrometry assay. This assay was used to analyze cardiac biopsy samples from one patient in the first cohort of the SUNRISE-FA trial before and after treatment with

1.8×10¹¹ vg/kg of LX2006 and two patients in the second cohort before and after treatment with 5.6×10¹¹ vg/kg of LX2006. We observed an increase in FXN levels as measured by LCMS relative to pre-treatment baseline levels in all three patients evaluated to date (n=3). These results are shown in the graphic below.



We observed an approximately five-fold increase in protein on average in the second dose cohort relative to the first cohort. We also observed dose-dependent response in our IND-enabling murine studies in the MCK model, where a non-linear relationship was observed between dose delivered and frataxin protein expression. This non-linear relationship is also supported by the results of a Lexeo-sponsored preclinical murine study conducted by Weill Cornell Medicine that was presented at ASGCT in 2023. In this study, a 3-fold increase in dose resulted in an approximately 5-fold increase in protein observed in the heart of MCK mice, and an additional 3-fold increase in dose resulted in an approximately 40-fold increase in protein. These increases in dose and protein expression each corresponded to greater improvements in survival and cardiac function. We believe these preclinical studies suggest that modest increases in dose have the potential to meaningfully increase frataxin in the heart and correspond to dose dependent improvements in efficacy.

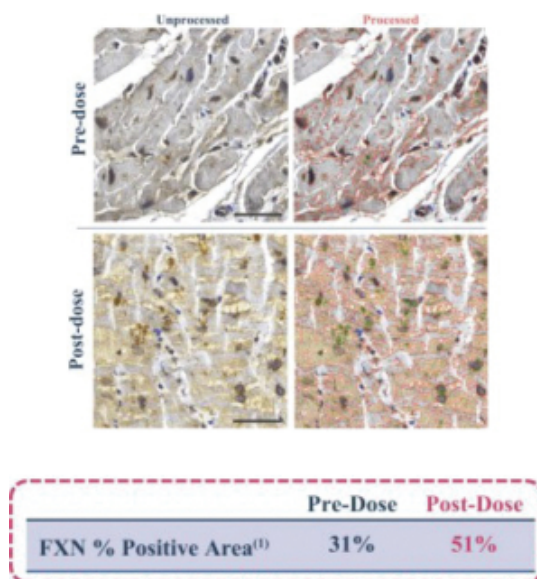
To our knowledge, SUNRISE-FA is the first clinical trial to evaluate frataxin levels in the target organ of FA cardiomyopathy patients. Average pre-treatment frataxin levels in the heart across the three subjects was 1.18ng/mg which we believe represents approximately two percent of frataxin in the heart relative to healthy controls based on a separate analysis from 29 cadaver tissue samples from 16 individuals. Previous published studies have identified frataxin levels in peripheral tissues (skin, buccal, PBMCs) ranging from approximately

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21% to 32% of normal individuals, but our research suggests FA patients likely have substantially lower levels of frataxin in the heart than in peripheral tissues. We believe the observed differences in baseline frataxin levels between peripheral tissues and the heart are likely due to substantial differences in mitochondrial content across these tissue types. As such, we believe that delivering even low levels of frataxin to the heart has the potential to restore mitochondrial function and may be sufficient for physiological improvement.

Additionally, one qualitative and two blinded quantitative immunohistochemistry, or IHC, assessments have been performed by three independent labs on biopsy samples taken from the cohort 1 patient. LCMS is a quantitative assessment of the amount of frataxin within the sample, whereas IHC is an anti-body based method in which frataxin protein expression can be visualized and then quantified, measured as a percent of total area stained. All three laboratories observed more FXN signal in the post-treatment sample compared to the pre-treatment sample. Blinded quantitative analysis demonstrated a 65% increase in FXN positive area in the post-treatment sample compared to the pre-treatment sample. The FXN area was calculated as a percentage of total tissue showing FXN expression which are denoted by red and green outline of lightly or darkly positive FXN area, respectively, in the images on the right. These results are shown in the graphics below. Furthermore, histological and transmission electron microscopy (TEM) assessments in this patient identified preserved tissue architecture without adverse safety signals or evidence of myocarditis.

Quantitative IHC Analysis



(1) Area measurement in square microns, FXN area as a percentage of total tissue / cardiomyocyte area.

In summary, these biopsy results reflect measurable increases in frataxin in the heart of three patients after treatment with LX2006. We believe that LX2006 is the first clinical-stage program for FA to show an increase in frataxin in cardiac tissue and this initial expression data, coupled with cardiac biomarker improvements observed in the Weill Cornell investigator initiated trial described below, demonstrate the potential of LX2006 to impact disease pathology at even the lowest dose cohort. We expect to report additional data from SUNRISE-FA in mid-2024.

Additional ongoing clinical trial of AAVrh10.hFXN (LX2006 at Lexeo)

AAVrh10.hFXN, which we refer to as LX2006, is currently being evaluated in an additional ongoing clinical trial conducted by Weill Cornell Medicine (NCT05302271). This is an investigator-sponsored, single-site

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study, and our trial (NCT05445323) is a multi-site trial sponsored by us. Both clinical studies use drug product manufactured at Weill Cornell Medicine utilizing the same process with identical doses in each study's cohort 1 and cohort 2. Safety information is being shared across these clinical studies.

In July 2023, researchers at Weill Cornell Medicine shared publicly, at a webinar sponsored by the Friedreich's Ataxia Research Alliance, preliminary interim data from cohort 1 of its ongoing study evaluating AAVrh10.hFXN in patients with FA cardiomyopathy. The two patients with six-month follow-up data after treatment both demonstrated declines in cardiac hypertrophy as measured by cardiac MRI analyzing left ventricular mass index, or LVMI. Increasing LVMI has been reported to be associated with progression and severity of FA cardiomyopathy. As shown in the table below, at the six-month timepoint a 10% decline in LVMI was observed in both treated patients with six-month follow-up data. Importantly, declines in cardiac hypertrophy, measured by decreases in LVMI, have been associated with clinical benefit in other cardiovascular diseases, including treatment for hypertension.

LVMI From First Two Patients Treated with 6-Month Data

LVMI	Baseline (g/m²)	6 Month (g/m²)	Nominal Change (g/m²)	% Change
Subject 1	109	98	-11	-10%
Subject 2	81	73	-8	-10%

Additionally, at the six-month timepoint a 74% and 9% decline in troponin I was demonstrated in the two subjects treated with follow-up data at that timepoint. Troponins are cardiac structural proteins which are released into the circulation following cardiomyocyte injury or necrosis. Troponin I is specific to cardiac tissue and is itself a measure of cardiac injury; thus, a reduction in troponin I suggests a reduction in cardiac injury.

High Sensitivity Troponin I From First Two Patients Treated with 6-Month Data

High Sensitivity Troponin I	Baseline (ng/L)	6 Month (ng/L)	Nominal Change (ng/L)	% Change
Subject 1	148	39	-109	-74%
Subject 2	224	203	-21	-9%

Additionally, one subject was evaluated through the six-month timepoint utilizing cardiopulmonary exercise testing, or CPET, which is an assessment of maximum oxygen consumption, or VO₂ max, and demonstrated an approximately 43% increase in VO₂ max versus baseline. We believe the totality of this interim data demonstrates the potential of LX2006 to halt or reverse disease progression as measured by improvements in LVMI, troponin I levels, and CPET performance in the patients treated in the Weill Cornell Medicine study.

LX2020 for the treatment of ACM caused by PKP2 mutations

We are developing LX2020 as an AAVrh10-based gene therapy for the treatment of ACM caused by mutations in the *PKP2* gene, or PKP2-ACM. LX2020 is designed to provide a functional *PKP2* gene to increase desmosomal PKP2 protein levels, reassemble desmosomes, and prevent cardiac arrhythmias and prevent or treat cardiac dysfunction. *PKP2* is the most frequently mutated desmosomal gene, and we believe there are more than

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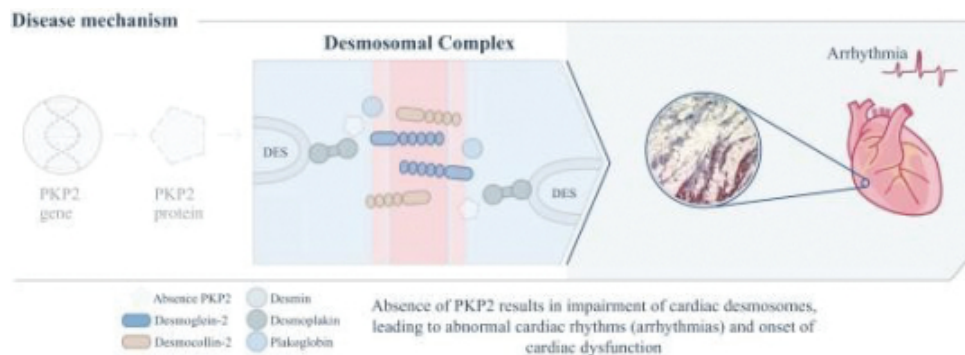
60,000 ACM patients affected by the *PKP2* mutation in the United States. We received IND clearance from the FDA for LX2020 in July 2023 and Fast Track and Orphan Drug designations from the FDA in December 2023. We expect to dose the first patient in the planned Phase 1/2 trial in the first half of 2024 and to provide an interim data readout from cohort 1 in the second half of 2024.

Overview of ACM

ACM is a genetic heart disease primarily characterized by myocardial cell loss and the replacement of heart muscle with fibrotic tissue and fatty deposits. ACM can result from mutations in several desmosomal genes. These genetic mutations impair the structure and function of cardiac desmosomes, which are membrane protein complexes engaged in cell-to-cell adhesion and the structural integrity of the ventricular myocardium. Lack of functioning cardiac desmosomes can lead to myocardial cell death and fibrosis, heart dysfunction, rhythm abnormalities, and sudden death. Standard of care may include antiarrhythmics, implantable cardioverter-defibrillators, and ablation procedures; however, none of these therapies address the underlying cause of myocardial dysfunction and ACM.

The figure below shows the role of the PKP2 protein in the formation of the desmosome.

Overview of ACM disease mechanism caused by deficient *PKP2* Gene



We estimate that ACM has a prevalence of approximately 130,000 patients in the United States and estimate that over half of all ACM patients have a genetic form of the disease with five desmosomal genes accounting for nearly all of the identified genetic causes of ACM. We have initially targeted the *PKP2* gene because mutations in this gene are the most commonly known genetic cause of ACM. We believe that mutations in the *PKP2* gene are associated with approximately 75% of all genetic cases of ACM, resulting in approximately 60,000 and 75,000 ACM patients affected by the *PKP2* mutation in the United States and European Union, respectively. Most familial cases of the disease have an autosomal dominant pattern of inheritance, meaning one copy of an altered gene in each cell is sufficient to cause the disorder. Since having only one functioning copy of the *PKP2* gene is insufficient to produce the wild-type phenotype, this results in a phenomenon in genetics known as haploinsufficiency.

Symptoms of the disease can include palpitations, dizziness, heart failure and sudden death. Abnormal function of the right ventricle, fatty or fibrotic infiltrates in the myocardium, abnormal electrocardiogram, or ECG, arrhythmias, or a family history of ACM can all lead physicians to diagnose the disease. No effective treatments for PKP2-ACM exist.

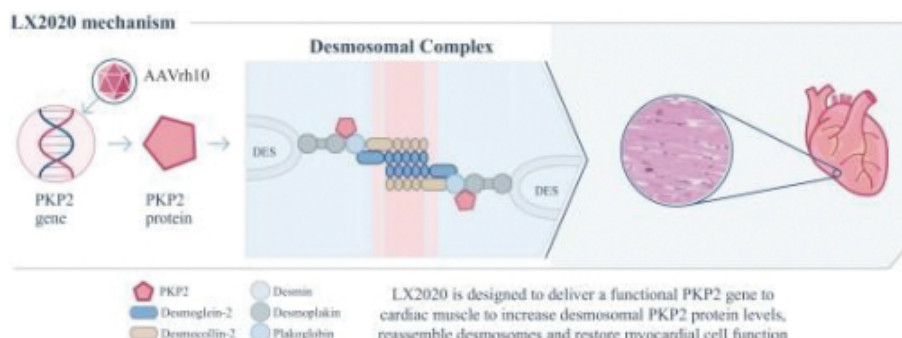
Our approach: LX2020

We are developing LX2020 as an AAVrh10-based gene therapy candidate for the treatment of PKP2-ACM. LX2020 is designed to intravenously deliver a functional *PKP2* gene to cardiac muscle to increase desmosomal

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PKP2 protein levels and restore myocardial cell function. We believe that by delivering a functional *PKP2* gene, LX2020 has the potential to address the underlying cause of PKP2-ACM for many patients and have a significant effect on lifespan by reassembling the cardiac desmosomes, preventing cardiac arrhythmias and preventing or treating cardiac dysfunction.

LX2020 is designed to deliver functional *PKP2* to restore normal desmosomal function



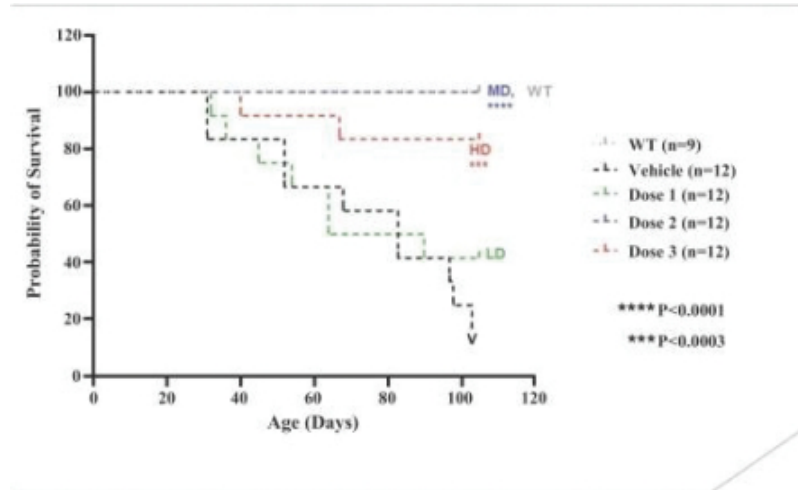
Preclinical studies

Our preclinical murine studies of LX2020 demonstrated improved cardiac structure and function, and the survival of mice harboring a pathologic human mutation in *PKP2*. The genetic mouse model we utilize in our preclinical studies is a CRISPR Cas9-edited model constitutively expressing a mutation in the *PKP2* gene found in humans with ACM. *PKP2* RNA splicing and protein levels are impacted in this model, which recapitulates all classic PKP2-ACM disease features. In a preclinical study of NHPs, LX2020 demonstrated a favorable safety profile with no safety signals.

Preclinical efficacy studies

We have completed preclinical studies demonstrating that delivery of LX2020 led to the reassembling of the cardiac desmosome, prevented arrhythmias and cardiac dysfunction, and increased survival in a genetic murine model of ACM harboring *PKP2* patient genetics. Three dose levels were administered to approximately three-week-old homozygous *PKP2* mice (a more severe disease murine model) with necropsy at twelve weeks post LX2020 treatment and eight-week-old *PKP2* heterozygous mice (a less severe murine model) with necropsy at eight weeks post LX2020 treatment. In the *PKP2* homozygous mouse study, 12 mice (six males and six females) in each group were treated with one of three dose cohorts (Dose 1 vg/kg, Dose 2 vg/kg, or Dose 3 vg/kg, which varied by approximately a half-log increase between each dose). As shown in the graphic below, within twelve weeks, approximately 90% of mice that received vehicle and 58% of mice that received Dose 1 vg/kg dose of LX2020 died. Conversely, 100% of mice that received the Dose 2 vg/kg dose and 83% of the mice that received the Dose 3 vg/kg dose survived until the 12-week necropsy time period. No *PKP2* protein expression was found in the mice that died in the Dose 3 vg/kg dose cohort, whereas all mice that survived in this cohort expressed *PKP2*. This and other characteristics of the mice indicate the potential of a technical injection dosing error.

LX2020 significantly extended survival in severe murine model



We also performed quantitative MRI analysis which showed dose-dependent improvement in cardiac function (end-diastolic volume, or EDV, end-systolic volume, or ESV, and ejection fraction, or EF) in *PKP2* homozygous mice, compared to vehicle-treated animals as shown in the graphic below.

Quantitative MRI analysis showed improvement in cardiac function in homozygous murine model

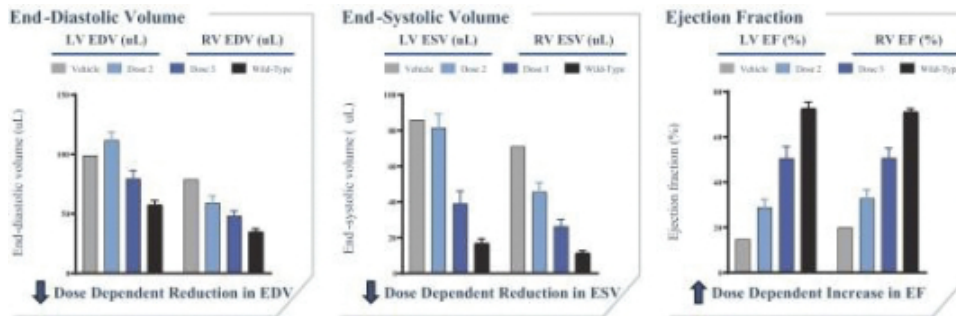
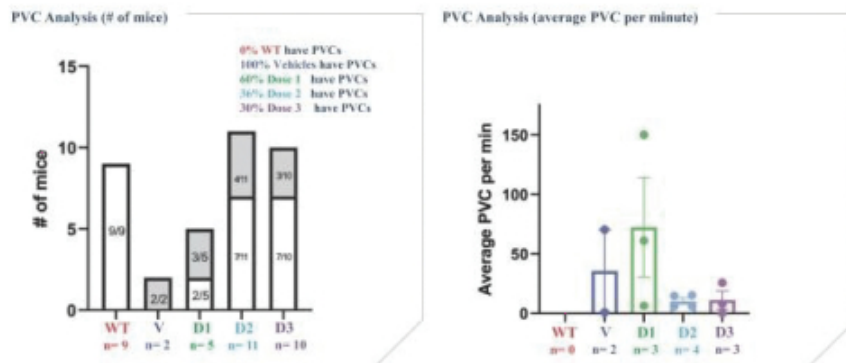


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As shown in the graphic below, we also completed surface ECG analyses which showed a reduction in arrhythmias as measured by premature ventricular contractions in *PKP2* homozygous murine models.

LX2020 treatment reduced premature ventricular contractions

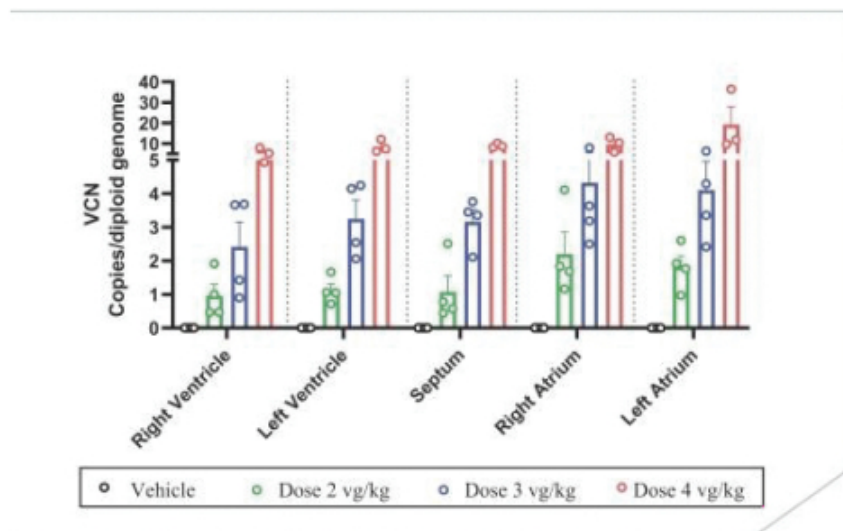


We also observed an 18% reduction in the QRS interval at Dose 3 vg/kg in the *PKP2* homozygous mouse model, indicating improvement in ventricular depolarization abnormalities. Additional dose-dependent improvements were detected through histological evaluations, revealing decreases in heart size, diminished right ventricular wall thinning, and reduced fibrosis and calcification.

Preclinical safety studies

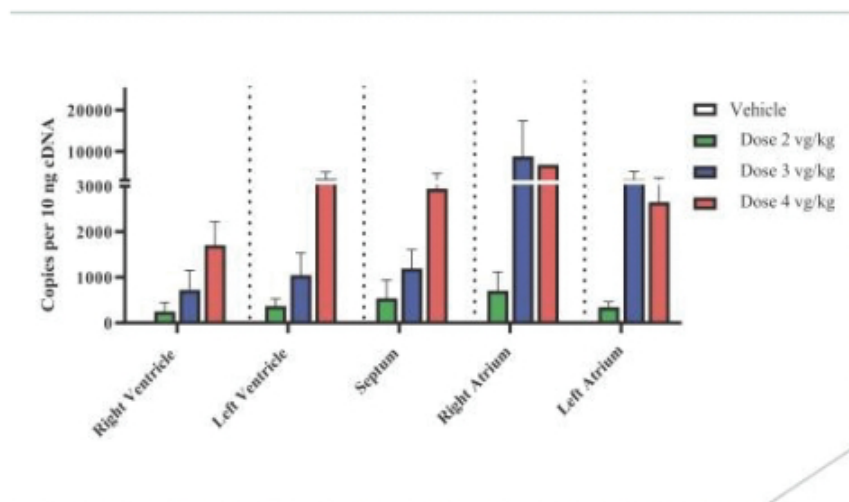
In a twelve week safety study of NHPs, three doses of LX2020 (Dose 2 vg/kg, Dose 3 vg/kg, or Dose 4 vg/kg, which vary by approximately a half-log increase between each dose) were administered to four monkeys per group (two males and two females) and resulted in dose-dependent increases in LX2020 biodistribution (reported as vector copy number, or VCN) in various regions of the heart as shown below. The biodistribution assay utilized qPCR technique with LX2020 transgene-specific primers.

VCN in various heart regions in NHPs



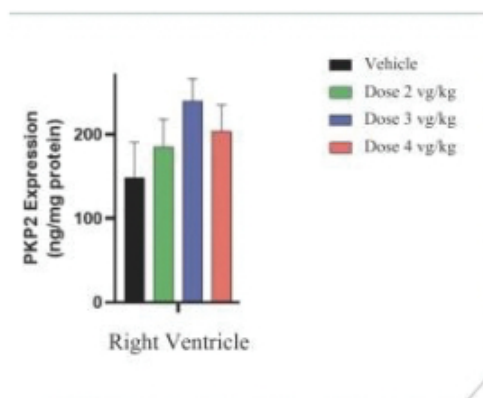
Additionally, in the NHP study, mRNA expression following LX2020 administration was assessed using an RT-qPCR assay with human *PKP2* transgene-specific mRNA primers. Data is reported as copies per nanogram of cDNA input, as shown below, and demonstrates that administration of LX2020 showed a dose-dependent increase of human *PKP2* mRNA in various regions of the heart.

***PKP2* mRNA expression (RT-qPCR) in NHPs**



Human *PKP2* protein expression was assessed in the NHP hearts using an electrochemiluminescence, or ECL, immunoassay. Notably, unlike the VCN and mRNA assays shown above, where LX2020-specific primers were synthesized to eliminate the detection of endogenous NHP DNA and mRNA, the commercially available antibodies used to detect human *PKP2* protein expressed by LX2020 also detect endogenous NHP *PKP2*. Given the high level of amino acid sequence similarity between the human and NHP *PKP2* proteins, generating anti-*PKP2* antibodies specific to one of the two species is impossible. Thus, the *PKP2* levels measured by the ECL assay represent the sum of endogenous NHP *PKP2* and exogenous human *PKP2* encoded by LX2020. Data are reported as ng of *PKP2* protein per mg of total protein.

***PKP2* protein expression in the right ventricle in NHPs**

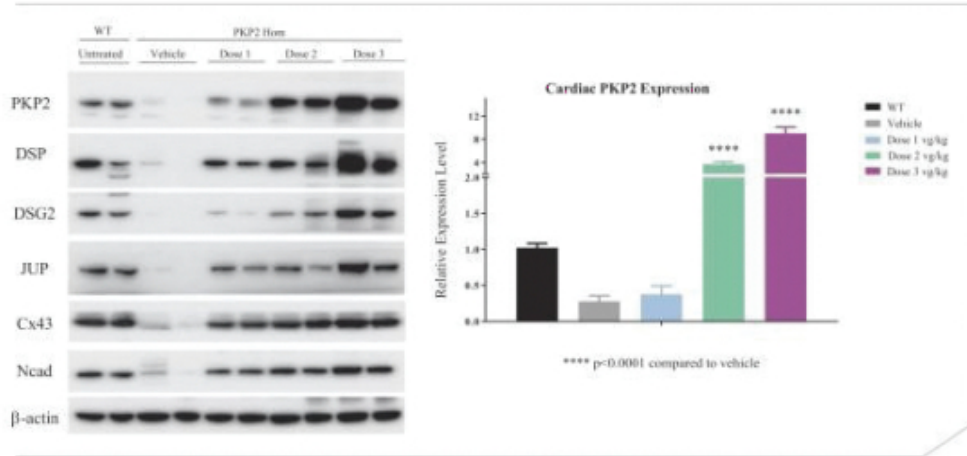


Whereas a dose-response level in DNA and RNA was observed in most heart regions in the NHPs treated with LX2020, protein levels did not correlate directly with dose. However, a dose-dependent increase in human

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PKP2 protein expression was seen in the *PKP2* homozygous mouse model as shown in the graphic below that translated well to cardiac activity. Three-week-old adult, *PKP2* homozygous mice were administered a single dose of LX2020 (Dose 1 vg/kg, Dose 2 vg/kg, and Dose 3 vg/kg) or formulation buffer (vehicle) intravenously and sacrificed twelve weeks post dose. LX2020 administration resulted in a dose-dependent increase in hPKP2 expression which was sufficient to prevent the cardiac cell-cell junction deficits of the classic proteins that make up the desmosome, including DSP, desmoglein-2, or DSG2, plakoglobin, or JUP, and N-cadherin, or Ncad, that have all been implicated in human ACM along with the gap junction protein connexin 43 (Cx43).

Cardiac PKP2 expression in homozygous mouse model



Possible explanations for the differences seen in mice and primate models include the following:

- *Inhibited Translation/Intracellular Protein Degradation:* Desmosomal proteins such as PKP2 are tightly regulated within striated muscle to maintain intracellular homeostasis. Therefore, excessive protein levels, such as those in wild-type primates, will likely result in decreased RNA translation and PKP2 protein degradation. This has been observed in previous studies and may explain why this effect was only observed in the wild-type primates but not in the murine model of PKP2 deficiency.
- *Interference from NHP endogenous PKP2 levels:* PKP2 protein is abundant in NHP cardiac muscle and can fluctuate considerably. Furthermore, for the most part, antibodies cannot distinguish between human and primate PKP2. Hence, the different protein levels in primates may be due to fluctuations in basal primate PKP2, not human PKP2 transcribed from LX2020.

Importantly, in the NHP safety study, all LX2020 doses (Dose 2 vg/kg, Dose 3 vg/kg, and Dose 4 vg/kg) demonstrated favorable safety profiles, including no abnormal effects on clinical signs, body weight, clinical pathology, hematology, histopathology, ECHOs, cardiac biomarkers, or ECG. Collectively these data indicate LX2020 was generally well tolerated during the twelve-week duration of the study in NHPs. The maximum dose of Dose 4 vg/kg is thus recognized as the NOAEL (no observed adverse event level) in NHPs.

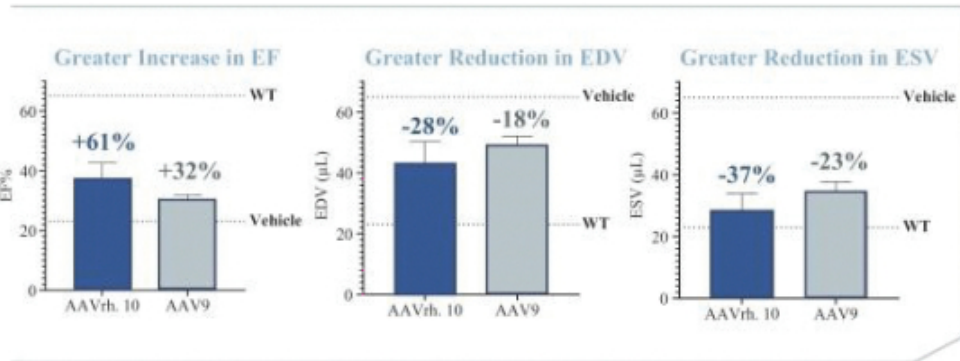
Preclinical Vector Comparability Studies

Additionally, we completed preclinical studies comparing AAVrh10 and AAV9 in head-to-head studies measuring cardiac function in a *PKP2* homozygous mouse model administered with human *PKP2*. Human *PKP2* was administered before disease onset on day two (neonatal administration) to five mice in each group, and

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cardiac function was measured by MRI. As shown in the graphic below, AAVrh10 demonstrated a trend of improvements in EF, EDV, and ESV compared to AAV9.

AAVrh10 showed greater trends of cardiac function improvement versus AAV9 in PKP2 mouse models



LX2020 clinical development and trial design

We received IND clearance from the FDA for LX2020 in July 2023 and Fast Track and Orphan Drug designations from the FDA in December 2023 for the treatment of PKP2-ACM. LX2020 is being evaluated in HEROIC-PKP2, a first-in-human, 52-week, open-label, dose-ascending multicenter trial to determine the safety and tolerability of LX2020 in patients with PKP2-ACM. Preliminary efficacy measures will include myocardial protein expression, biomarkers measuring cardiac structure and function, and arrhythmia burden. Key inclusion criteria include: patients aged 18-65 years with a confirmed diagnosis of ACM with either 2010 Task Force Criteria or 2020 International Criteria for ACM; documented PKP2 mutation; existing implantable cardioverter defibrillator (ICD) that is MRI compatible; and a minimum threshold of PVCs over a 24 hour period. LX2020 will be administered as a one-time intravenous infusion to patients in at least two ascending-dose cohorts of three patients each, with the potential for cohort expansion. Prednisone and rapamycin will be utilized for immune suppression. The starting dose will be 2×10^{13} vg/kg, which was selected based on the dose-response seen in our preclinical studies. A second cohort will be evaluated with a dose of 6×10^{13} vg/kg. There will be a long-term follow-up for patients who receive LX2020 to monitor ongoing safety for a total of five years, per FDA requirement. We have initiated clinical trial startup activities and expect to dose the first patient in the first half of 2024 and expect to provide an interim data readout from cohort 1 in the second half of 2024.

Additionally, we have initiated SNAPSHOT-PKP2, a natural history study designed to evaluate PKP2-ACM disease progression up to two years retrospectively and over twelve months prospectively. The study will enroll up to 20 patients in the United States.

Additional cardiovascular gene therapy programs: LX2021 and LX2022

We believe there are additional targets in genetically defined cardiac disease that have the potential to be addressed through AAV-based gene therapies. We plan to continue to innovate with novel capsids, promoters, and delivery methods to optimize our early-stage assets.

LX2021 for the treatment of DSP cardiomyopathy

Overview

LX2021 is a gene therapy candidate we are developing to intravenously deliver the coding sequence for the functional Cx43 protein for a group of inherited cardiac muscle disorders associated with a high risk of sudden

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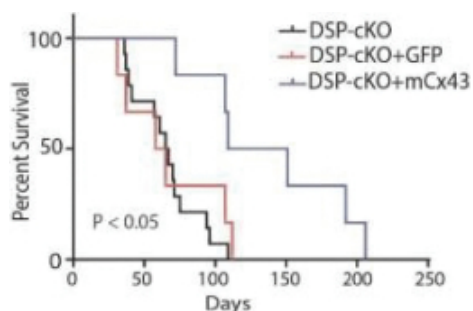
death, including ACM and certain forms of dilated cardiomyopathy, which are typically due to abnormalities or deficits in cardiac desmosomes. Cx43 is an integral protein component of gap junctions, and functionally allows small molecules and ions to flow directly between cells to allow for electrical synchronization of muscle contraction. In patients with heart disease, including heart failure, Cx43 is often relocalized in the lateral walls of myocardial cells. As a result, it is significantly reduced at cardiac muscle cell junctions, especially in ACM populations. Restoring Cx43 protein to cardiac muscle cell-cell junctions can potentially treat multiple genetic causes of ACM because the cardiac loss of Cx43 is a molecular deficit generally observed in all ACM patient populations. DSP is a structural protein critical for force transmission in heart muscle. Mutations in DSP cause a distinct form of ACM and a certain form of dilated cardiomyopathy, known as DSP cardiomyopathy. Unlike many forms of ACM that predominantly affect the right side of the heart, DSP cardiomyopathy frequently affects the left side of the heart. The prevalence of DSP cardiomyopathy is unknown but may be as high as 4% of all inherited dilated cardiomyopathies and 4% of ACM, impacting up to 35,000 individuals in the U.S. Given the role of Cx43 in other forms of heart failure, we are also evaluating LX2021 for the treatment of additional indications beyond DSP cardiomyopathy.

We have conducted a preclinical study in a cardiac-specific DSP loss-of-function mouse model, or DSP-cKO mice, which is a genetic model of ACM. DSP-cKO mice develop molecular, histological and physiological features of ACM and ultimately die prematurely from heart failure. In this study, we observed that severely diseased mice treated with AAV-based gene therapy expressing Cx43 display fewer arrhythmias, have improved cardiac mechanical function, and experience an almost two-fold increase in lifespan when compared to untreated DSP-cKO adult mice. We plan to initiate IND-enabling studies for LX2021 in 2024.

Preclinical studies

Our preclinical studies have demonstrated that mice harboring DSP cardiomyopathy experience a nearly two-fold increase in lifespan when treated with an AAV-based gene therapy candidate expressing Cx43 compared to controls. A single dose of an AAV-based gene therapy candidate representing Cx43 was administered intravenously to the cardiac-specific DSP-cKO mice, at four to six weeks of age. DSP-cKO mice are a genetic model of ACM harboring severe structural disease and loss of Cx43. RNA analysis of the treated mice revealed that cardiac-specific Cx43 gene delivery increased Cx43 RNA levels. Additionally, the Cx43-treated DSP-cKO mice showed a re-expression of mechanical junction proteins in their hearts compared to controls. Essential basement membrane proteins, including N-cadherin, as well as desmosomal proteins, PKP2 and junction plakoglobin, were also restored to cell-cell junctions in Cx43-treated DSP-cKO hearts compared to end-stage untreated DSP-cKO hearts, which had limited localization of junctional proteins at cell-cell junctions. These findings further validate the ability of Cx43 to resurrect the cardiac mechanical muscle junction complex in diseased hearts.

Administration of AAV-based therapy expressing Cx43 improved survival in severe ACM mouse model



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Based on these findings, we believe that restoration of Cx43 has the potential to positively impact cardiomyocyte physiology and lifespan in the DSP-cKO mice and provide direction for our ongoing development efforts, including the evaluation of other vector serotypes. We plan to initiate IND-enabling studies for LX2021 in 2024.

LX2022 for the treatment of HCM caused by TNNI3 mutations

Overview

LX2022 is a gene therapy candidate we are developing to intravenously deliver a functional *TNNI3* gene to myocardial cells to treat a distinct form of HCM due to mutations in the *TNNI3* gene. With an estimated prevalence of 1 in 500 people in the United States, HCM is one of the most common forms of genetic cardiomyopathy caused by mutations that affect the cardiac sarcomere in approximately 75% of cases. It is estimated that as many as 25,000 patients in the United States and 34,000 patients in the European Union are affected by HCM caused by mutations in the gene *TNNI3*. The *TNNI3* gene encodes troponin I, an essential protein in the thin filament of the sarcomere, which is involved in cardiac contraction and relaxation. Mutations in the gene often result in left ventricular hypertrophy and restrictive cardiomyopathy, leading to arrhythmias and heart failure. We plan to complete candidate selection for LX2022 in 2024.

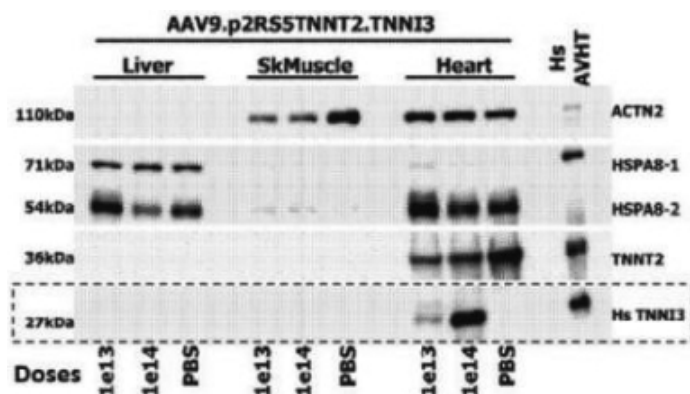
Overview of HCM caused by TNNI3 Mutations

HCM is a genetic disorder characterized by left ventricular hypertrophy and is one of the most common forms of genetic cardiomyopathy. Mutations in over a dozen genes encoding the thick and thin filaments of the cardiac sarcomere have been linked to HCM. *TNNI3*, a critical component of the thin filament of the sarcomere, accounts for approximately 5% of all HCM cases. Genetic mutations may affect any portion of the sarcomere, including thin and thick filaments, ultimately leading to cardiac muscle dysfunction. While thick filament mutations are the most common and best characterized, thin filament mutations remain clinically relevant. Some evidence suggests thin filament mutations are associated with more arrhythmias and earlier progression to advanced heart failure due to a disease mechanism distinct from other forms of inherited cardiomyopathy. In addition, *TNNI3* mutations are less likely to cause cardiac obstruction and hence less likely to benefit from surgical (myectomy) and medical interventions (myosin inhibitors) targeting obstruction.

Preclinical Studies

Our preclinical data from an A157V *TNNI3* murine model, a model which results in cardiac dysfunction, was generated to validate the delivery of an AAV-based therapy candidate expressing human *TNNI3* regulated by a cardiac-specific promoter. At four weeks post injection, the level and specificity of *TNNI3* gene expression were evaluated in the liver, skeletal muscle, and heart tissue. Endogenous TNNT2 protein expression indicates cardiomyocyte-specific tissue, whereas ACTN2 indicates expression in only myocytes. HSPA8-1 and HSPA8-2 were blotted as housekeeping gene controls. As *TNNI3* was regulated by a cardiac-specific promoter, expression of *TNNI3* was only detected in the heart tissue, as shown in the figure below.

TNNI3 Expression in Liver, Skeletal Muscle, and Heart Tissue in A157V Mouse Model



Additionally, retro-orbital injection of AAV9-hTNNI3 expressing A157V mutation was performed on three-month-old mice euthanized four weeks later. The hearts were dissected and immunostained. Immunostaining revealed robust expression of human *TNNI3* in the cardiomyocyte after treatment with AAV-based *TNNI3*.

Moreover, high magnification imaging demonstrated the incorporation of *TNNI3* within the cardiac sarcomere. Based on these preclinical findings, we believe that AAV-based therapy expressing *TNNI3* can be administered to achieve transgene expression in heart tissue. Our ongoing development efforts include evaluation of other vector serotypes. We have also developed a novel CRISPR CAS9-edited novel porcine model of HCM that resembles the human phenotype. This model will allow us to perform preclinical testing in a system to resemble the human condition and perform physiological assays to evaluate efficacy.

APOE4-associated Alzheimer's disease programs

Alzheimer's disease is a progressive neurodegenerative disorder that is the leading cause of cognitive decline in late adult life. The Alzheimer's Association estimates that there were 6.2 million patients living in the United States with Alzheimer's disease in 2021, with costs to the nation exceeding \$350 billion. Aging of the population is expected to significantly increase the socioeconomic burden of this disease in the coming decades, and the Alzheimer's Association further estimates that as many as 12.4 million patients in the United States could have the disease by 2050.

Our Alzheimer's disease portfolio

In a similar theme to the cardiovascular treatment landscape, we see an opportunity of a comparable magnitude in precision therapies treating Alzheimer's disease. There is only one fully approved drug (lecanemab) that has demonstrated slowing of disease progression in patients with early Alzheimer's disease. However, within the prevalent population of 6.2 million patients in the United States suffering from Alzheimer's

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disease, there are number of subpopulations, including *APOE4* patients who comprise approximately 2-3% of the U.S. population but comprise approximately 15% of Alzheimer's disease patients. We believe they represent a unique subpopulation within the broader disease space. In clinical trials of lecanemab and donanemab, a different safety profile (a higher incidence of amyloid-related imaging abnormalities, or ARIA) was observed for *APOE4* homozygous patients compared to other patient genotypes. In fact, the lecanemab label has a warning for ARIA and states that the risk is higher in *APOE4* homozygous patients. Additionally, in both clinical trials, *APOE4* homozygous patients demonstrated lower efficacy results compared to heterozygotes and noncarriers in terms of a reduction in clinical dementia rating. We believe this points to the unmet need within the *APOE4* population and also raises questions of whether there is a unique disease pathology that *APOE4* genetics confer on Alzheimer's disease patients. Because of this differentiated treatment effect, we believe precision therapies, particularly those focused on the underlying genetics of Alzheimer's disease, may have a substantial impact on this treatment landscape.

We are building a portfolio of approaches aimed at treating the genetics underlying Alzheimer's disease. In our lead Alzheimer's disease program, LX1001, we are initially targeting homozygous *APOE4*-associated Alzheimer's disease patients by administering AAVrh10 containing the *APOE2* gene. Our approach to treating Alzheimer's disease is predicated on the belief that expressing the protective *APOE2* in the CNS of *APOE4* homozygous patients will halt or slow the progression of Alzheimer's disease. We believe these patients represent an ideal target for gene therapy because the *APOE4* homozygous profile is the most common genetic driver of Alzheimer's disease. We plan to leverage biomarkers for LX1001 to potentially enable us to rapidly validate proof-of-mechanism and support further development efforts. We believe that positive clinical data from our ongoing Phase 1/2 trial has the potential to validate our novel approach to treating the *APOE4* sub-group and can help inform the development plan of our next generation candidates LX1021 and LX1020, which we believe could demonstrate an even more dramatic effect to slow or halt the progression of Alzheimer's disease. Importantly, we believe gene therapy allows for a unique approach to treating the genetics of Alzheimer's disease by delivering a therapeutic which acts upstream of both the amyloid- β and tau-driven pathology of Alzheimer's disease. This treatment strategy is designed to impact multiple pathways, as opposed to most other treatments in development which target a single mechanism of Alzheimer's disease.

Our current gene therapy programs under development for the treatment of Alzheimer's disease are:

LX1001: an AAVrh10-based gene therapy candidate that is designed to express the protective *APOE2* protein in the CNS of *APOE4* homozygous patients. In December 2022 we reported that three patients from the first dose cohort demonstrated a consistent trend towards improvement in CSF tau biomarkers at the 12-month visit and also observed expression of the protective *APOE2* protein in all patients in the first dose cohort with follow-up data.

LX1021: an AAVrh10-based gene therapy candidate that is designed to express the Christchurch-modified *APOE2* protein in the CNS of *APOE4* homozygous patients. The Christchurch mutation has been observed to protect patients against Alzheimer's disease even in the presence of significant amyloid pathology.

LX1020: an AAVrh10-based gene therapy candidate that is designed to express the protective *APOE2* protein in the CNS of *APOE4* homozygous patients, while concurrently delivering miRNA to suppress the expression of the *APOE4* protein.

We are developing LX1001 to target homozygous *APOE4*-associated Alzheimer's disease patients, the highest risk population to develop Alzheimer's disease and the sub-group we believe will be the most likely to demonstrate a potential treatment effect from *APOE2*-based gene therapy. We completed enrollment in the trial in the fourth quarter of 2023 and expect to report additional interim data from all cohorts in the Phase 1/2 trial in the second half of 2024.

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Background of Alzheimer's disease

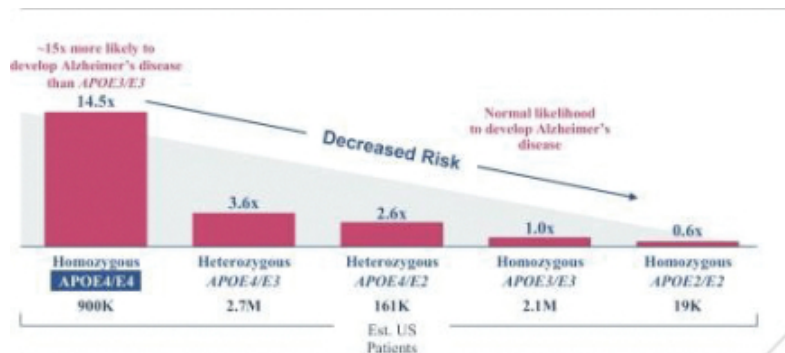
Alzheimer's disease is characterized by a complex underlying pathology in the CNS, including accumulation of A β plaques, abnormal phosphorylation of tau, development of tau tangles, inflammation, and progressive loss of neurons, all of which combine to precipitate a progressive decline in cognitive function. APOE, a lipid transport protein, is the major transporter of cholesterol in the brain and is involved in synaptic integrity and plasticity, glucose metabolism, and cerebrovascular function.

Extracellular amyloid beta plaques and tau neurofibrillary tangles are the principle pathological hallmarks of Alzheimer's disease. Therapeutics that modulate plaques and tangles historically have been the focus of substantial research and development efforts; however, there is currently no treatment for Alzheimer's disease that is approved as disease modifying. The FDA granted accelerated approval to the anti-amyloid antibody intravenous infusion therapies aducanumab (Aduhelm) and lecanemab (Leqembi) for the treatment of patients with either the mild cognitive impairment or mild dementia stage of Alzheimer's disease. These recent approvals demonstrate that the FDA has utilized an accelerated approval pathway for Alzheimer's disease therapies.

Background on homozygous APOE4-associated Alzheimer's disease

Presence of APOE4 is the most common genetic risk factor for Alzheimer's disease. The prevalent APOE alleles are APOE4, APOE3 and APOE2, with the E4 allele increasing risk and reducing the age of onset and the E2 allele decreasing risk and markedly delaying the age of onset. APOE4 homozygous patients, individuals who have two copies of the E4 allele, are at the highest risk and are approximately fifteen times more likely to develop Alzheimer's disease than the general population. It is estimated that approximately 60% of Alzheimer's disease patients carry at least one APOE4 allele as compared with approximately 25% of age-matched and healthy controls. The APOE3 allele is believed to have a neutral impact on disease progression.

Alzheimer's disease risk by APOE genotype (by odds ratio)



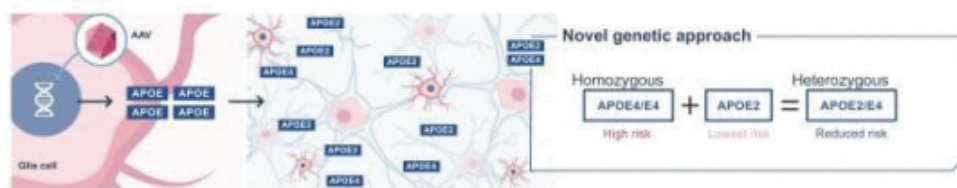
Third-party genetic epidemiology data from humans suggest that APOE4 and APOE2 are co-dominant, or both expressed, such that APOE2/E4 heterozygous individuals have a substantially reduced risk of developing the disease as compared to APOE4/E4 homozygous individuals. Therefore, the expression of APOE2 appears to significantly offset the deleterious effects of the APOE4 allele.

We believe that administering the APOE2 gene to APOE4 homozygous patients is a promising approach because it has the potential to address several pathways that are involved in the progression of the disease.

Our approach: LX1001

LX1001 is an AAVrh10-based gene therapy candidate for the treatment of APOE4 homozygous patients. It is designed to deliver the human APOE2 gene into the CSF to halt or slow the progression of Alzheimer's disease.

LX1001 is designed to address the genetic driver of Alzheimer's disease by delivering *APOE2* gene into CNS of *APOE4* homozygous patients



Based on what is known regarding gene therapies delivered to the CNS, we believe LX1001 will only require a single dose because neurons are post-mitotic, or incapable of further cell division, so there will be no dilution of extra-chromosomal LX1001 caused by cell division. APOE is a secreted protein; thus, only a fraction of neurons needs to be transduced with LX1001 to secrete what we anticipate to be potentially efficacious levels of the protective isoform. We have designed LX1001 so that the protective isoform has the potential to be widely available to cells in the brain and able to compensate for non-transduced cells.

The FDA granted Fast Track designation to LX1001 for the treatment of *APOE4* homozygous patients with Alzheimer's disease to slow disease progression. We plan to seek other regulatory designations and consider accelerated clinical development pathways based on biomarker data that may establish early proof-of-mechanism and is expected to predict clinical benefit.

Clinical development

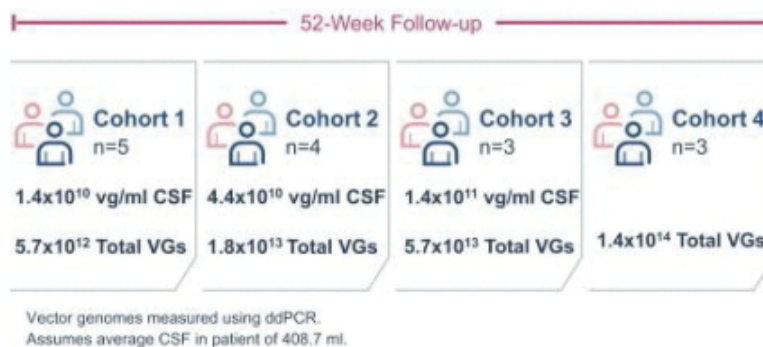
Trial design

We are evaluating LX1001 in an open-label, dose-ranging Phase 1/2 clinical trial in patients who are *APOE4* homozygous patients with clinical diagnoses ranging from mild cognitive impairment to mild or moderate dementia due to Alzheimer's disease. All patients have evidence of amyloid plaque by PET scan and/or CSF biomarkers consistent with Alzheimer's disease.

The primary objective of the trial is to evaluate the safety of LX1001 administered to the CNS via injection between cervical vertebrae 1 and 2, or intracisternal injection, and to establish the maximum tolerated dose. The trial is also designed to evaluate the conversion of the CSF from the *APOE4* homozygous profile to an *APOE4/E2* profile. Additional secondary endpoints include CSF biomarkers, including AB42, total tau, and phosphorylated tau, amyloid PET scan, structural MRI imaging and cognitive tests.

As shown below, the clinical trial is a dose-ranging trial of LX1001 in four ascending dose cohorts (1.4×10¹⁰ vg/ml, 4.4×10¹⁰ vg/ml, 1.4×10¹¹ vg/ml and 1.4×10¹⁴ total genomes), with the dose for each patient in the first three cohorts determined based on CSF volume measured by MRI and patients in the fourth cohort receiving a fixed dose. Each dose cohort will consist of approximately three to five patients for a total of approximately 15 patients in the trial. Key patient enrollment criteria include *APOE4* homozygous genetic profile, patient age of 50 years or older, positive amyloid PET scan (for cohorts 1-3), evidence of CSF biomarkers consistent with Alzheimer's disease, and diagnosis that ranges from mild cognitive impairment to mild or moderate dementia due to Alzheimer's disease. Corticosteroids are utilized for immune suppression. There will be a long-term follow-up for patients who receive LX1001 to monitor ongoing safety for a total of five years, per FDA requirement.

Design of Phase 1/2 clinical trial of LX1001



Preliminary trial results

Preliminary data from cohort 1, which was presented at the 2022 Clinical Trials on Alzheimer’s Disease conference, showed an increase in CSF APOE2 levels, in all patients with follow-up data at three months or longer and such levels remained generally stable through the 12-month visit. These results, shown below, demonstrate that the *APOE2* transgene is being expressed in the CNS in all patients treated with LX1001 and signal the potential for treatment to convert the *APOE4* homozygous profile to a combined *APOE2/APOE4* profile.

Interim APOE2 CSF expression levels (fmol/mL)(1)

	Patient No.	Dose (Total gc) ⁽²⁾	Month 0	Month 3	Month 6	Month 12
Cohort 1	101-1001	6.7E+12	0.0	0.3	0.4	0.4
	101-1002	7.9E+12	0.0	--	--	0.1
	101-1004	5.0E+12	0.0	0.8	--	--
	101-1005	6.8E+12	0.0	0.3	0.3	0.3

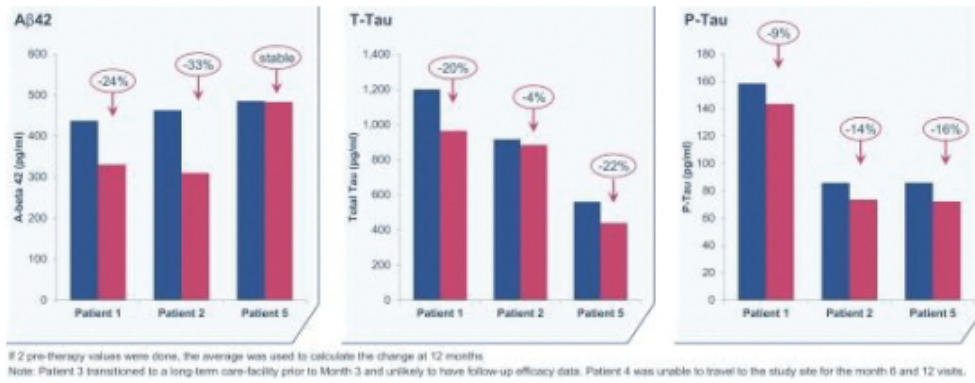
(1) APOE2 quantified by liquid chromatography mass spectroscopy. Research grade assay; preliminary results represent the average of all runs for each sample (1-5 runs / sample).

(2) Cohort 1 patient doses were administered using a nominal LX 1001 titer based on qPCR. Values represent actual total dose administered using LX1001 titer by ddPCR

Note: Patient 101-1003 transitioned to a long-term care-facility prior to Month 3 and unlikely to have follow-up efficacy data. Patient 101-1004 was unable to travel to the study site for the month 6 and 12 visits.

Additionally, in the three patients from cohort 1 with data available at 12-months post treatment, we observed consistent trends towards improvement (reduction) in the core CSF biomarkers T-tau and P-tau that are associated with neurodegeneration and Alzheimer’s disease specifically and we have also seen declines or stabilization of Ab42, the beta-amyloid peptide associated with Alzheimer’s disease as shown below. We believe this interim data reflects evidence of proof-of-mechanism and supports our unique approach to treating the genetics of Alzheimer’s disease by delivering a therapeutic which acts upstream of both the amyloid-b and tau-driven pathology of Alzheimer’s disease.

Interim CSF core biomarker data in Cohort 1 patients with 12-month data



Natural history data for T-tau and P-tau would suggest that on average these biomarkers should increase over the course of the year, versus the decline that we observed following treatment. We completed enrollment in the trial in the fourth quarter of 2023 and expect to report additional interim data from all cohorts in the Phase 1/2 trial in the second half of 2024.

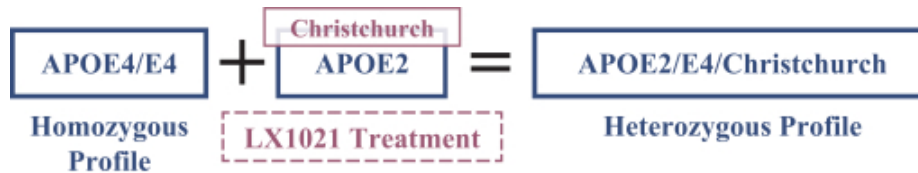
Next-generation gene therapy solutions for Alzheimer’s disease: LX1021 and LX1020

We believe that positive clinical data from our ongoing Phase 1/2 clinical trial of LX1001 has the potential to validate our novel approach to treating the *APOE4* sub-group and can help inform the development plan of our next-generation candidates, LX1021 and LX1020.

LX1021 for the treatment of *APOE4* homozygotes

LX1021: We are designing LX1021 to treat *APOE4* homozygous patients by adding a Christchurch mutation-modified *APOE2* to the CNS. The Christchurch mutation has been recognized to protect individuals against Alzheimer’s disease even in the presence of significant amyloid pathology. The mechanism of this protection may relate to the fact that APOE, in the presence of the Christchurch mutation, binds poorly to heparan sulfate proteoglycans (HSPGs), molecules found on the surface of neurons, which may inhibit the spread of tau pathology between cells. We believe this approach has the potential to enhance the protective effect of APOE2 in homozygous *APOE4*-associated Alzheimer’s disease. We plan to hold a pre-IND meeting with the FDA in 2024 to provide direction for our ongoing development efforts.

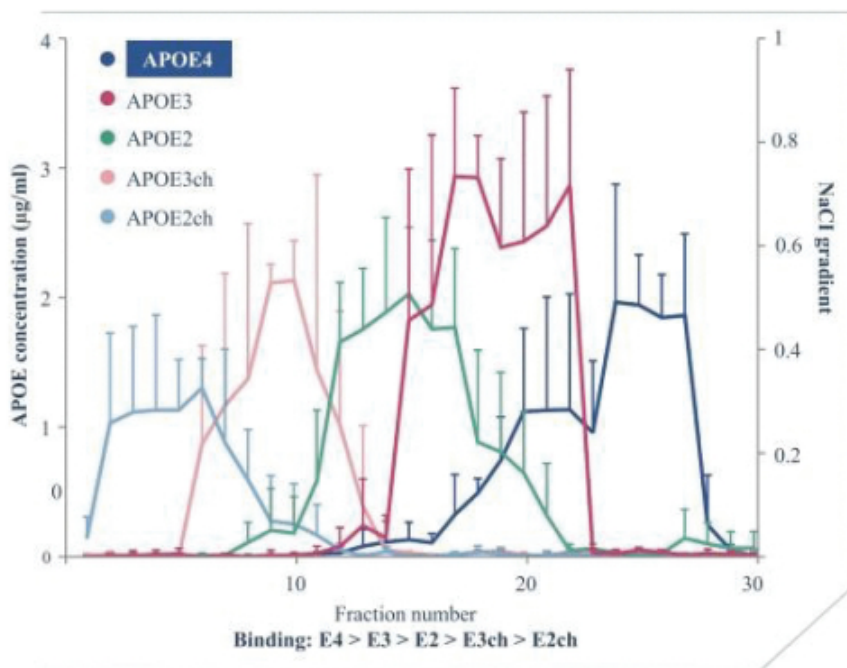
LX1021 is designed to add a Christchurch mutation-modified *APOE2* gene to the CNS of *APOE4* homozygous patients



Preclinical Studies

In vitro experiments conducted by other researchers and replicated at Weill Cornell Medicine have demonstrated that Christchurch mutation-modified *APOE3* behaves similarly to the *APOE2* allele in that both isoforms bind poorly to heparan sulfate proteoglycans, or HSPGs. As shown in the figure below, differences in one to two amino acids drive the major physiological differences between the three *APOE* isoforms, including differences in protein structure and regulation of amyloid- β aggregation, in addition to binding affinities to HSPGs. This increased binding to HSPGs is implicated in the propagation of tau tangles and uptake of toxic forms of tau and amyloid- β aggregation.

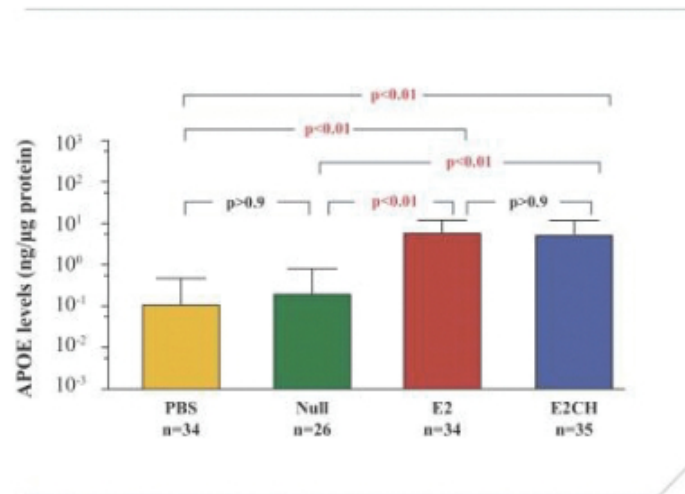
Binding affinity of different *APOE* alleles and Christchurch mutation-modified *APOE3* and *APOE2*



Interim data from preclinical studies have demonstrated that ApoE4-driven amyloid and tau pathology can be suppressed by AAV-mediated delivery of Christchurch-modified *APOE2* to the CNS in well-established murine Alzheimer's disease models (amyloid mice, APP.PS1/TRE4 and tau mice, P301S/TRE4). In both mouse models, 2×10^{10} total genomes were administered directly into the hippocampus.

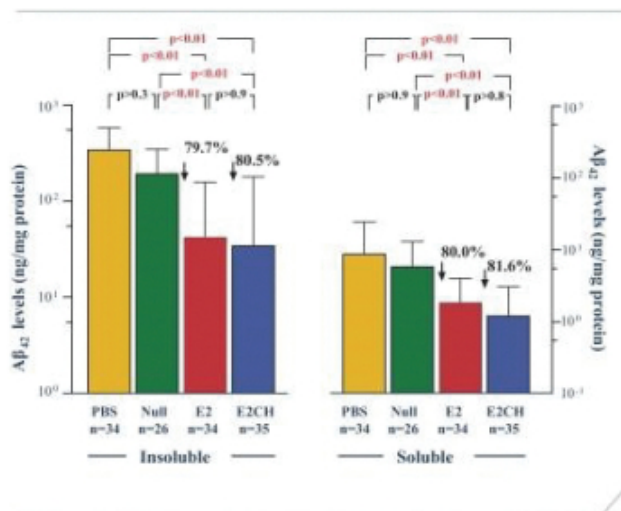
In the APP.PSEN1/TR4 model AAVrh.10hApoE2 (LX1001) and AAVrh.10hApoE2Ch (LX1021) were administered to 2.5-month old mice and resulted in significant production of ApoE2 mRNA and protein expression over phosphate buffered saline, or PBS, and null-treated animals, with no significant differences between the two constructs.

APOE protein Levels (Amyloid Mice)



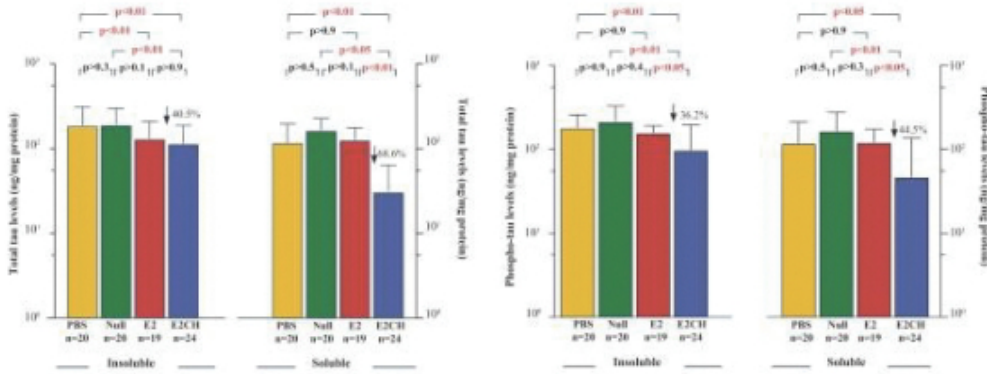
Both constructs resulted in significant reductions in insoluble and soluble Aβ₄₂ and Aβ₄₀ levels, with no significant differences between the two constructs. Additionally, both constructs demonstrated improvement in various behavioral tests compared to PBS or null-treated animals, with no significant differences between the two constructs. These results demonstrate that LX1021 may have similar therapeutic potential as LX1001 as both treatments suppress the amyloid pathology in the APP.PSEN1/TRE4 mice.

Insoluble and Soluble Aβ₄₂ Levels (Amyloid Mice)



In the P301S/TRE4 model, both LX1001 and LX1021 were administered to 5.5-month old mice and resulted in significant production of APOE2 mRNA and protein expression over PBS and null-treated animals, with no significant differences between the two constructs, a similar finding to the amyloid mouse model. However, when evaluating total tau and p-tau, pathological markers of Alzheimer's disease that are impacted in the P301S/TRE4 model, we observed significant improvement in the LX1021 treated mice compared to the LX1001 treated mice.

Tau Mice, Total Tau Levels (Left) and Phosphorylated Tau Levels (Right)



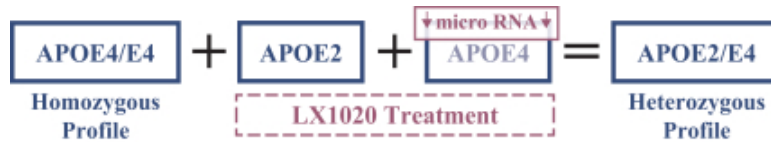
Behavioral tests, including Y maze, Barnes Test, and novel object recognition showed LX1001 treatment demonstrated some behavioral improvement compared to PBS-treated animals but no significant behavioral improvement compared to null-treated animals. However, LX1021 treatment resulted in significant behavioral improvement on all assessments compared to PBS and null-treated animals, as well as significant behavioral improvement on all assessments compared to LX1001.

We believe the data generated to date from two mouse models demonstrate that LX1021 has the potential to impact both amyloid and tau pathology, the core pathological features of Alzheimer’s disease, potentially providing for enhanced treatment effect compared to the wild-type E2 transgene. We plan to continue preclinical development efforts and hold a pre-IND meeting with the FDA in 2024 to provide direction for our ongoing development efforts.

LX1020 for the treatment of APOE4 homozygotes

LX1020: We are designing LX1020 to treat *APOE4* homozygous patients by delivering both the protective *APOE2* allele and miRNA to suppress *APOE4*. We believe delivery of *APOE2* with concurrent suppression of *APOE4* will achieve a higher degree of conversion to the *APOE4/E2* heterozygous profile, which should lead to greater therapeutic effect.

LX1020 is designed to add *APOE2* gene while suppressing *APOE4*



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Preclinical Studies

In preclinical studies, AAV9 vectors expressing APOE-targeting miRNAs (AAV-mirAPOE) was administered to the hippocampus of TRE4 mice, a mouse model with murine *APOE* gene deleted and replaced with human *APOE4*. When compared with an AAV9-mCherry (a control vehicle), the AAV-mirAPOE downregulated hippocampal APOE4 mRNA levels by 2.8-fold, suggesting that an AAV-mediated gene therapy incorporating APOE-targeting miRNA into the expression cassette may potentially reduce human *APOE4* in the CNS. These findings demonstrate that AAV-based delivery of APOE-targeting miRNA may be able to serve as part of a therapeutic approach for treating *APOE4* homozygous Alzheimer's patients and provide direction for our ongoing development efforts. We plan to complete candidate selection for LX1020 in 2024.

Competition

The biotechnology and pharmaceutical industries are characterized by rapidly changing technologies, significant competition and a strong emphasis on intellectual property. This is also true for the development and commercialization of treatments for cardiovascular and neurodegenerative diseases such as FA, PKP2-ACM, and Alzheimer's disease and broadly across gene therapies. While we believe that our management and scientific team's deep expertise in gene therapy provides us with competitive advantages, we face competition from several sources, including large and small biopharmaceutical companies, government agencies and academic and private research institutions. Not only must we compete with other companies that are focused on gene therapy technology, but any product candidates that we successfully develop and commercialize will compete with existing therapies, to the extent applicable, and new therapies that may become available in the future.

Drug development, particularly in the gene therapy field, is highly competitive and subject to rapid and significant technological advancements. A significant unmet medical need exists in each of the indications that we are targeting, and it is likely that additional drugs will become available in the future for the treatment of these diseases.

We are aware that our competitors are developing product candidates for the treatment of diseases that our product candidates will target. With respect to LX2006, we are aware of preclinical gene therapy programs in development at Solid Biosciences Inc. and Lacerta Therapeutics, Inc. and those being developed in collaborations between Voyager Therapeutics, Inc. and Neurocrine Biosciences, Inc. Additionally, we are aware that Prime Medicine, Inc. and Tune Therapeutics, Inc. have early-stage gene editing discovery efforts. Among other treatment modalities for FA, we are aware that Larimar Therapeutics, Inc. is developing a clinical stage product candidate, CTI-1601, that Design Therapeutics, Inc. is developing a product candidate, DT-216P2, and that Reata Pharmaceuticals, Inc.'s omaveloxolone (Skyclarys) was approved by the FDA in 2023. In 2023 Biogen Inc. acquired Reata Pharmaceuticals, Inc. for approximately \$7.3 billion and is currently commercializing Skyclarys.

With respect to LX2020, both Rocket Pharmaceuticals Inc., or Rocket, and Tenaya Therapeutics Inc. are developing an AAV-based gene therapy candidate designed to deliver a functional *PKP2* gene to patients with PKP2-ACM.

With respect to our portfolio of gene therapy programs for the treatment of homozygous APOE4-associated Alzheimer's disease, we are aware that uniQure, N.V. is pursuing AMT-240, a preclinical gene therapy candidate for autosomal dominant Alzheimer's disease intended to silence the APOE4 variant while expressing a protective variant and Novartis has a gene therapy candidate for Alzheimer's disease which is in the early preclinical stages of development. Many large and small pharmaceutical companies and academic institutions are developing potential treatments for the condition given the significant unmet need and the large population suffering from Alzheimer's disease. There are multiple FDA-approved treatments for Alzheimer's disease, including donepezil (Aricept), memantine (Namenda), and in July of 2023, lecanemab (Leqembi) was approved by the FDA for the treatment of Alzheimer's disease based on the observed reduction of amyloid beta plaque. In addition, Eli Lilly and Company's product candidate for the treatment of Alzheimer's disease, donanemab, has completed a Phase 3 clinical trial. Finally, we are aware that Voyager Therapeutics, Inc. has a program targeting Alzheimer's disease.

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Many of our existing or potential competitors have substantially greater financial, technical and human resources than we do and significantly greater experience in the discovery and development of product candidates, as well as in obtaining regulatory approvals of those product candidates in the United States and in foreign countries.

These competitors also compete with us in recruiting and retaining qualified scientific and management personnel and establishing clinical trial sites and patient registration for clinical trials, as well as in acquiring technologies complementary to, or necessary for, our programs. Mergers and acquisitions in the pharmaceutical and biotechnology industries may result in even more resources being concentrated among a smaller number of our competitors. Smaller or early-stage companies may also prove to be significant competitors, particularly through collaborative arrangements with large and established companies.

We will face competition from other drugs or from other non-drug products and treatments currently approved or that will be approved in the future in the cardiovascular and neurology field, including for the treatment of diseases and diseases in the therapeutic categories we intend to target. Therefore, our ability to compete successfully will depend largely on our ability to:

- develop, manufacture and commercialize drugs that are superior to other products in the market;
- demonstrate through our clinical trials that our product candidates are differentiated from existing and future therapies;
- attract qualified scientific, product development and commercial personnel;
- obtain patent or other proprietary protection for our medicines;
- obtain required regulatory approvals;
- obtain coverage and adequate reimbursement from, and negotiate competitive pricing with, third-party payors; and
- successfully collaborate with pharmaceutical companies in the discovery, development and commercialization of new medicines.

The availability of our competitors' products could limit the demand, and the price we are able to charge, for any product candidate we develop. The inability to compete with existing or subsequently introduced drugs would have an adverse impact on our business, financial condition and prospects. In addition, the reimbursement structure of currently approved or future gene therapies by other companies could impact the anticipated reimbursement structure of our gene therapies, if approved, and our business, financial condition, results of operations and prospects.

Established pharmaceutical companies may invest heavily to accelerate discovery and development of novel compounds or to in-license novel compounds that could make our product candidates less competitive. In addition, any new product that competes with an approved product must demonstrate compelling advantages in efficacy, convenience, tolerability and safety in order to overcome price competition and to be commercially successful. Accordingly, our competitors may succeed in obtaining patent protection, discovering, developing, receiving regulatory and marketing approval for, or commercializing, drugs before we do, which would have an adverse impact on our business and results of operations.

Intellectual property

We actively seek to protect our proprietary technology, inventions, and other intellectual property that is commercially important to the development of our business by a variety of means, such as seeking, maintaining, and defending patent rights, whether developed internally or licensed from third parties. We also may rely on trade secrets and know-how relating to our proprietary technology platform, on continuing technological

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innovation and on in-licensing opportunities to develop, strengthen and maintain the strength of our position in the field of gene therapy that may be important for the development of our business. We also intend to seek patent protection or rely upon trade secret rights to protect other technologies that may be used to discover and validate targets, and that may be used to manufacture and develop novel gene therapy products. We are a party to license agreements that give us rights to use specific technologies in our gene therapy candidates and in manufacturing our products. Additional regulatory protection may also be afforded through data exclusivity, market exclusivity and patent term extensions where available.

As of March 27, 2024, we in-licensed two U.S. patents, ten pending U.S. non-provisional patent applications, four pending U.S. provisional applications, one pending PCT applications, six foreign patents and 61 pending foreign applications.

In regard to our LX2006 product candidate, as of March 27, 2024, we in-license from Cornell University and Adverum Biotechnologies, Inc., or Adverum, two U.S. patents, two pending U.S. non-provisional patent applications, five foreign patents granted in India, Mexico, South Africa, and New Zealand and eight pending foreign applications pending in such jurisdictions as Australia, Brazil, Canada, Eurasia, Europe, Israel and Mexico. Not accounting for any patent term adjustment and assuming that all annuity and/or maintenance fees are paid timely, these patents, and patent applications if granted, will expire in 2033. These patents and pending patent applications disclose and/or contain composition-of-matter claims to an AAV vector encoding *FXN*, or a fragment thereof, and disclose and/or contain claims to methods of producing and methods of treatment using the AAV *FXN* vector. Cornell University co-owns these patents and patent applications with Institut National de la Santé et de la Recherche Médicale (INSERM), Centre National de la Recherche Scientifique (CNRS), Université de Strasbourg, Université Paris-Saclay and Assistance Publique-Hôpitaux de Paris (APHP).

We have rights to one pending PCT application that relates to our LX2006 cardiac FA program. Not accounting for any patent term adjustment and assuming that all annuity and/or maintenance fees are paid timely, if granted, patent applications claiming benefit of this PCT application, will expire in 2043. The PCT application discloses and/or contains composition-of-matter claims to a pharmaceutical dosage form of viral vectors encoding *FXN*, and discloses and/or contains claims to methods of producing and methods of treatment using the pharmaceutical dosage form.

In regard to our LX2020 product candidate, we in-license from The Regents of the University of California one pending U.S. non-provisional patent application and 17 pending foreign applications pending in such jurisdictions as Australia, Brazil, Canada, China, Colombia, Eurasia, Europe, Hong Kong, Israel, India, Japan, Korea, Mexico, New Zealand, Singapore and South Africa. Not accounting for any patent term adjustment and assuming that all annuity and/or maintenance fees are paid timely, if granted, these patent applications, will expire in 2042. This PCT application discloses and/or contains composition-of-matter claims to a vector encoding human plakophilin-2 (*PKP2*), and discloses and/or contains claims to methods of producing and methods of treatment using the PKP2 vector.

In regard to our LX2021 product candidate, we in-license from The Regents of the University of California two pending U.S. non-provisional patent applications, one foreign patent granted in Europe, and six pending foreign applications pending in Australia, Canada, China, Europe, Israel, and Japan. Not accounting for any patent term adjustment and assuming that all annuity and/or maintenance fees are paid timely, if granted, these patent applications will expire in 2038. These pending patent applications disclose and/or contain composition-of-matter claims to a vector encoding a human connexin 43 (Cx43) polypeptide, and disclose and/or contain claims to methods of producing and methods of treatment using the Cx43 vector.

In regard to our LX2022 product candidate, we in-license from The Regents of the University of California one pending U.S. non-provisional patent application. Not accounting for any patent term adjustment and assuming that all annuity and/or maintenance fees are paid timely, if granted, this patent application, and patent applications claiming priority to this patent application, will expire in 2040. This pending U.S. non-provisional

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patent application discloses and/or contains composition-of-matter claims to a vector encoding a human *TNNI3* gene, and discloses and/or contains claims to methods of producing and methods of treatment using the *TNNI3* vector.

In regard to our LX1020 product candidate, we in-license from Cornell University one pending U.S. non-provisional application and 14 pending foreign applications pending in Australia, Brazil, Canada, China, Colombia, Europe, Hong Kong, Israel, Japan, Korea, Mexico, New Zealand, Singapore and South Africa. Not accounting for any patent term adjustment and assuming that all annuity and/or maintenance fees are paid timely, if granted, these patent applications will expire in 2040. The pending patent applications disclose and/or contain composition-of-matter claims to a vector encoding human APOE2 protein and encoding one or more RNAi nucleic acid sequences for inhibition of APOE4 mRNA, and disclose and/or contain claims to methods of producing and methods of treatment using the APOE2+/APOE4- vector.

In regard to our LX1021 product candidate, as of March 27, 2024 we in-license from Cornell University three pending U.S. provisional applications, one pending U.S. non-provisional application, and 13 pending foreign applications pending in Australia, Brazil, Canada, China, Europe, Hong Kong, Israel, Japan, Korea, Mexico, New Zealand, Singapore and South Africa. Not accounting for any patent term adjustment and assuming that all annuity and/or maintenance fees are paid timely, if granted, these patent applications will expire in 2040. The pending patent applications disclose and/or contain composition-of-matter claims to a vector encoding a mutated human apolipoprotein E protein, and disclose and/or contain claims to methods of producing and methods of treatment using the mutated human apolipoprotein E vector.

In regard to our LX1001 product candidate, we co-own with Cornell University one pending U.S. provisional application and one pending PCT application. Not accounting for any patent term adjustment and assuming that all annuity and/or maintenance fees are paid timely, if granted, patent applications claiming the benefit of this PCT application will expire in 2043. This pending patent application discloses and/or contains composition-of-matter claims to a pharmaceutical dosage form of viral vectors encoding human APOE2, and discloses and/or contains claims to methods of producing and methods of treatment using the pharmaceutical dosage form.

License, research collaboration and sponsored research agreements

First license agreement with Cornell University

In May 2020, we entered into a license agreement with Cornell University, or the Cornell First License Agreement, pursuant to which we obtained a sublicensable, worldwide license under certain patents to make, use, and sell products that are covered by a valid claim of a licensed patent, are based on the transferred IND from Cornell University, use any licensed materials, or are produced using a licensed method, and to practice certain licensed technology, in all cases, for all human and non-human prophylactic and therapeutic uses. In July 2022, we entered into Amendment No. 1 to the Cornell First License Agreement that updated fees and royalties payable by us under the Cornell First License Agreement. In September 2022, we entered into Amendment No. 2 to the Cornell First License Agreement that clarified patent prosecution and maintenance obligations and added certain inventions and patents to the list of inventions and patent rights covered under the Cornell First License Agreement. In February 2024, we entered into Amendment No. 3 to the Cornell First License Agreement which added three pending U.S. provisional patent applications to the list of patent rights covered under the Cornell First License Agreement. The technology under the license includes portfolios for APOE, Alzheimer's disease, and Anti-Tau, although our license is not restricted by such indications. The license is exclusive with respect to certain patents and non-exclusive with respect to other patents. Additionally, under the Cornell First License Agreement, Cornell University assigned to us an IND for the use of AAVrh10.hAPOE2 vector to treat *APOE4* homozygous patients who are at risk of having or have Alzheimer's disease. Cornell University reserved the rights to publish and disseminate information about inventions included in the licensed technology and licensed patents, and to use, and allow other nonprofit institutions to use, the patents and technology for educational and research purposes.

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We are obligated to diligently proceed with the development, manufacture, and sale of licensed products, to raise certain amounts within specified time frames, to achieve certain development milestones within specified time frames, to meet agreed minimum-spend requirements, and to meet other diligence obligations. If we fail to perform these obligations, Cornell University could terminate the Cornell First License Agreement in full or in part in specified circumstances, subject to certain rights for us to extend these time frames.

Under the Cornell First License Agreement, we paid Cornell University an upfront payment of approximately \$0.15 million and entered into a purchase agreement for approximately \$0.6 million of convertible preferred securities. We are obligated to pay Cornell University an annual license maintenance fee ranging from the low four digits to the mid five digits, increasing annually until such time that we are commercially selling a licensed product. Under the Cornell First License Agreement, we are obligated to pay Cornell University up to \$8.4 million for each portfolio upon the achievement of specific clinical and regulatory milestones. We are obligated to pay Cornell University a flat royalty in the mid-single-digits based on net sales of a licensed product in a country, which royalty rate increases by one percent if the licensed product is an orphan drug, subject to a reduction upon the expiration of valid claims in licensed patents and certain reductions for third-party licenses. In addition, in certain specific instances where sales are made by a sublicensee, the royalty rate increases by an amount in the low to mid-single digits. If the royalties are below certain agreed amounts, we are required to pay Cornell University minimum annual royalties ranging from low-six digits to low-seven digits. The royalty term continues for each licensed product on a country-by-country basis beginning on the first commercial sale of such licensed product and ending on the latest of (a) expiration or invalidation of the last valid claim in the licensed patent, (b) the expiration of regulatory exclusivity, and (c) the month of the first commercial launch of a generic equivalent in such country. We are also obligated to pay Cornell University a percentage of sublicensing fees in the low-double digits to mid-double digits.

We may terminate the Cornell First License Agreement or any portfolio thereunder at any time upon ninety (90) days' advance written notice to Cornell University. Cornell University may terminate the Cornell First License Agreement if we commit a material breach and fail to cure such breach within a specified cure period after written notice or if we challenge the validity of a licensed patent. Upon expiration of the royalty term of a given licensed product in a country, the license becomes non-exclusive and royalty-free. Upon termination of the Cornell First License Agreement, all licenses and rights granted by either party will terminate, although we will have a period of time to sell off any remaining licensed product.

Second license agreement with Cornell University

In May 2020, we entered into a second license agreement with Cornell University, or the Cornell Second License Agreement, pursuant to which we obtained a sublicensable, worldwide license under certain patents to make, use, and sell products that are covered by a valid claim of a licensed patent, are based on the transferred IND from Cornell University, use any licensed materials, or are produced using a licensed method, and to practice certain licensed technology, in all cases, for all human and non-human prophylactic and therapeutic uses. In January 2022, we entered into Amendment No. 1 to the Cornell Second License Agreement, whereby we paid Cornell a material transfer fee and a license fee totaling \$30,000, and Cornell added certain materials to the list of original materials subject to the Cornell Second License Agreement. In July 2022, we entered into Amendment No. 2 to the Cornell Second License Agreement that updated fees and royalties payable by us under the Cornell Second License Agreement. The technology under the license includes a portfolio for FA, although our license is not restricted by such indications. The license is exclusive with respect to certain patents and non-exclusive with respect to other patents. Cornell University reserved the rights to publish and disseminate information about inventions included in the licensed technology and licensed patents, and to use, and allow other nonprofit institutions to use, the patents and technology for educational and research purposes.

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We are obligated to diligently proceed with the development, manufacture, and sale of licensed products, to raise certain amounts within specified time frames, to achieve certain development milestones within specified time frames, to meet agreed minimum-spend requirements, and to meet other diligence obligations. If we fail to perform these obligations, Cornell University could terminate the Cornell Second License Agreement in full or in part in specified circumstances, subject to certain rights for us to extend these time frames.

Under the Cornell Second License Agreement, we paid Cornell University an upfront payment of approximately \$0.15 million and entered into a purchase agreement for approximately \$0.6 million of convertible preferred securities. We are obligated to pay Cornell University an annual license maintenance fee ranging from the low four digits to the mid five digits, increasing annually until such time that we are commercially selling a licensed product. Under the Cornell Second License Agreement, we are obligated to pay Cornell University up to \$4.3 million for two portfolios and up to \$0.6 million for a third portfolio upon the achievement of specific clinical and regulatory milestones. Upon submitting our IND application for LX2006 to the FDA in the first quarter of 2022, we achieved the first clinical milestone under the Cornell Second License Agreement, and we paid \$0.1 million to Cornell University in the second quarter of 2022 in connection with this milestone. We are obligated to pay Cornell University a flat royalty in the mid-single-digits based on net sales of a licensed product in a country, which royalty rate increases by one percent if the licensed product is an orphan drug, subject to a reduction upon the expiration of valid claims in licensed patents and certain reductions for third-party licenses. In addition, in certain specific instances where sales are made by a sublicensee, the royalty rate increases by an amount in the low to mid-single digits. If the royalties are below certain agreed amounts, we are required to pay Cornell University minimum annual royalties ranging from low six digits to low seven digits. The royalty term continues for each licensed product on a country-by-country basis beginning on the first commercial sale of such licensed product and ending on the latest of (a) expiration or invalidation of the last valid claim in the licensed patent, (b) the expiration of regulatory exclusivity, and (c) the month of the first commercial launch of a generic equivalent in such country. We are also obligated to pay Cornell University a percentage of sublicensing fees ranging in the low double digits.

We may terminate the Cornell Second License Agreement or any portfolio thereunder at any time upon ninety (90) days' advance written notice to Cornell University. Cornell University may terminate the Cornell Second License Agreement if we commit a material breach and fail to cure such breach within a specified cure period after written notice or if we challenge the validity of a licensed patent. Upon expiration of the royalty term of a given licensed product in a country, the license becomes non-exclusive and royalty-free. Upon termination of the Cornell Second License Agreement, all licenses and rights granted by either party will terminate, although we will have a period of time to sell off any remaining licensed product.

Research collaboration agreement with Weill Cornell Medicine

In February 2021, we entered into a Research Collaboration Agreement with Cornell University on behalf of Weill Cornell Medicine, or the Cornell Collaboration Agreement, in connection with the Cornell First License Agreement and the Cornell Second License Agreement entered into in May 2020, collectively, the Cornell License Agreements. In February 2022, we entered into Amendment No. 1 to the Research Collaboration Agreement. The Cornell Collaboration Agreement, as amended, is referred to as the Amended Cornell Collaboration Agreement. Under the Amended Cornell Collaboration Agreement, we committed to fund scientific research at Weill Cornell Medicine related to the technology licensed to us pursuant to the Cornell License Agreements. Cornell University reserved the rights to publish and disseminate information about the results, excluding any inventions, generated from Cornell University's investigator's conduct of the research.

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Under the Cornell License Agreements, we committed to provide funding for research projects at Cornell University for a three-year period, with a minimum annual funding commitment of \$1.0 million. With respect to each Weill Cornell Medicine invention and joint invention and related joint results that is either an improvement to, or is dominated by, the patent rights under the Cornell License Agreements or is specifically designed for a licensed product under the Cornell License Agreements, or Improvement, and for which we have made an election to obtain such inventions, the Cornell License Agreements will be amended to include license grants to such Improvements following payment of the relevant amendment fee in the low five digits. With respect to each Weill Cornell Medicine invention and joint invention, and related joint results that are not Improvements, we have the first option to negotiate for a royalty-bearing, worldwide license under such intellectual property to develop, make, have made, use, offer for sale, sell, have sold and import products on commercially reasonable terms.

The Amended Cornell Collaboration Agreement expired in accordance with its terms in February 2024, and we have not entered into a new research collaboration agreement with Cornell University.

License agreement with Adverum

In January 2021, we entered into a license agreement with Adverum, which we amended in February 2022 pursuant to the First Amendment to the Adverum Agreement, such agreement as amended, the Amended Adverum Agreement. Pursuant to the Amended Adverum Agreement, we obtained an exclusive, sublicensable, worldwide license under certain patents, know-how, and other intellectual property relating to viral vector technology for gene therapy applications for the treatment of FA cardiomyopathy.

We are responsible for the development, manufacture, and commercialization of gene therapy products that consist of a specific nucleic acid sequence that is delivered by a specific gene therapy, or the Products. We are obligated to use commercially reasonable efforts to develop, obtain regulatory approval for, and commercialize the Products.

Under the Amended Adverum Agreement, we paid Adverum a \$7.5 million upfront payment. We are obligated to pay Adverum up to \$17.5 million upon the achievement of specified development and regulatory milestones, including a \$3.5 million development milestone that was achieved in the first quarter of 2023, and up to \$49 million in commercialization and sales milestones for the Products. We are obligated to pay Adverum tiered royalties ranging from high single-digits to sub teens based on annual aggregate worldwide net sales of Products, subject to reductions upon the expiration of valid claims in licensed patents and third-party licenses. The royalty term continues for each Product on a country-by-country basis beginning on the first commercial sale of such Product and ending on the latest of (a) expiration of the last valid claim in the licensed patent that covers the manufacture, use, or sale of the Product in such country, (b) the expiration of all regulatory and data exclusivity in such country, and (c) ten years after the first commercial sale of such Product in such country.

The Amended Adverum Agreement will expire, unless earlier terminated, on the expiration of the last royalty term for a Product in a particular country. We have the right to terminate the Amended Adverum Agreement at any time upon one-hundred twenty days' advance written notice to Adverum. In addition, subject to certain conditions, either we or Adverum may terminate the Amended Adverum Agreement upon the insolvency of the other or if the other party commits a material breach of the agreement and fails to cure such breach within a specified cure period after written notice is provided. Additionally, Adverum may terminate the Amended Adverum Agreement if we challenge the validity of any licensed patents. Upon expiration of the Amended Adverum Agreement, the license becomes royalty-free, irrevocable and perpetual. Upon termination of the Amended Adverum Agreement, all licenses, sublicenses, and rights granted by either party will terminate.

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First license agreement with the Regents of University of California, San Diego

Stelios, which we acquired in August 2021, is successor-in-interest to ARVC Therapeutics, Inc., or ARVC Therapeutics, under a worldwide license agreement entered into by ARVC Therapeutics and The Regents of The University of California on April 23, 2020, or the UCSD First License Agreement, pursuant to which they obtained a license to certain intellectual property related to gene therapies for ARVC. The UCSD First License Agreement relates to our development efforts for our LX2021 program. Pursuant to the UCSD First License Agreement, we obtained an exclusive, sublicensable, worldwide license under certain patents to make, use, sell, offer for sale and import services, methods, composition and products that incorporate or are developed using any licensed invention or licensed methods, or is covered by a valid claim of a licensed patent, or a Product, in all cases for all diagnostic and therapeutic uses. UCSD reserved the right to practice the relevant invention and licensed patents for educational and research purposes, to publish and disseminate information about the relevant invention and licensed patents, and to allow other nonprofit institutions to use, publish, or disseminate information about the relevant inventions and licensed patents for educational and research purposes.

Under the UCSD First License Agreement, we are obligated to achieve certain development milestones within specified time frames, to use commercially reasonable efforts to diligently develop, manufacture, and sell Products, and to meet agreed minimum-spend requirements. If we fail to perform any of these obligations, UCSD could either terminate the UCSD First License Agreement or convert the exclusive license to a nonexclusive license, subject to certain rights for us to extend these time frames.

The UCSD First License Agreement required Stelios to pay one-time up-front non-refundable cash fees of \$20,000. We are obligated to pay annual license maintenance fees in the mid-four digits to low-five digits, increasing until such time that we are commercially selling a Product. We are also obligated to pay up to \$4.75 million upon the achievement of specific development and commercialization milestones for the first Product and low-to mid-single digit royalties based on aggregate net sales, subject to certain reductions for third-party licenses. The royalty term continues until the expiration of the UCSD First License Agreement. Under the UCSD First License Agreement, if the royalties are below certain agreed amounts, we are required to pay UCSD minimum annual royalties ranging from low- to mid-five digits. We are also obligated to pay UCSD a percentage of sublicensing fees ranging in the low double digits. In the event that we assign the UCSD First License Agreement, we will be obligated to pay an assignment fee that will be determined based on certain aspects of the assignment.

The UCSD First License Agreement will expire, unless earlier terminated, on the expiration of the last claim of any licensed patent or patent applications in a particular country. We have the right to terminate the UCSD First License Agreement at any time upon sixty days' written notice to UCSD. UCSD may terminate the UCSD First License Agreement if we commit a material breach, if we fail to meet certain specified milestones within the prescribed time periods, if we are delinquent on any report or payment or provide an intentionally misleading report, if we fail to diligently develop and commercialize the licensed products, or if we challenge the validity of a licensed patent, in each case only if we fail to cure such problem within a specified cure period after written notice. Upon termination of the UCSD First License Agreement, all licenses and rights granted by UCSD to us will terminate.

Second license agreement with the Regents of University of California, San Diego

Stelios, which we acquired in August 2021, entered into a worldwide license agreement in August 2020 with The Regents of The University of California, or the UCSD Second License Agreement, pursuant to which they obtained a license to certain intellectual property and materials related to gene therapies for HCM. The UCSD Second License Agreement relates to our development efforts for our LX2022 programs. Pursuant to the UCSD Second License Agreement, we obtained an exclusive, sublicensable, worldwide license under certain patents to make, use, sell, offer for sale and import services, methods, composition and products that incorporate or are developed using any licensed invention or licensed methods or is covered by a valid claim of a licensed

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patent, or a Product, and a non-exclusive license to use nonpublic technical information, or Technology, in all cases for all diagnostic and therapeutic uses. UCSD reserved the rights to practice the relevant invention, Technology and licensed patents for educational and research purposes, to publish and disseminate information about the relevant invention, Technology and licensed patents, and to allow other nonprofit institutions to use, publish, or disseminate information about the relevant invention, Technology and licensed patents for educational and research purposes.

Under the UCSD Second License Agreement, we are obligated to achieve certain development milestones within specified time frames and to use commercially reasonable efforts to diligently develop, manufacture, and sell Products. If we fail to perform any of these obligations, UCSD could either terminate the UCSD Second License Agreement or convert the exclusive license to a nonexclusive license subject to certain rights for us to extend these time frames.

The UCSD Second License Agreement required Stelios to pay one-time up-front non-refundable cash fees of \$20,000. We are obligated to pay annual license maintenance fees in the mid-four digits to low-five digits, increasing until such time that we are commercially selling a Product. We are also obligated to pay up to \$2.4 million upon the achievement of certain development and commercialization milestones for the first Product and low-single digit royalties based on aggregate net sales, subject to certain reductions for third-party licenses. The royalty term continues until the expiration of the UCSD Second License Agreement. We are also obligated to pay UCSD a percentage of sublicensing fees ranging in the low double digits.

The UCSD Second License Agreement will expire, unless earlier terminated, on the expiration of the last claim of any licensed patent or patent applications in a particular country. We have the right to terminate the UCSD Second License Agreement at any time upon sixty days' written notice to UCSD. UCSD may terminate the UCSD Second License Agreement if we commit a breach, if we fail to meet certain specified milestones within the prescribed time periods, if we are delinquent on any report or payment, if we are not diligently developing or commercializing Products in accordance with our diligence obligations, if we provide any intentionally false report, or if we challenge the validity of a licensed patent, in each case only if we fail to cure such problem within a specified cure period after written notice. In the event that we assign the UCSD Second License Agreement, we will be obligated to pay an assignment fee that will be determined based on the acquisition price. Upon termination of the UCSD Second License Agreement, all licenses and rights granted by UCSD to us will terminate.

Third license agreement with the Regents of University of California, San Diego

In October 2021, we entered into a worldwide license agreement with UCSD, or the UCSD Third License Agreement, and collectively with the UCSD First License Agreement and the UCSD Second License Agreement, The UCSD License Agreements, pursuant to which we obtained a license to materials and intellectual property related to a gene therapy for ARVC. The UCSD Third License Agreement relates to our development efforts for our LX2020 program. Pursuant to the Third UCSD Agreement, we obtained an exclusive, sublicensable, worldwide license under certain patents to make, use, and sell, offer for sale and import services, methods, composition and products that incorporate or are developed using any licensed invention or licensed methods, or is covered by a valid claim of a licensed patent, or a Product, and an exclusive license to use nonpublic technical information, or Technology, in all cases for all diagnostic and therapeutic uses. UCSD reserved the rights to practice the relevant invention, Technology and licensed patents for educational and research purposes, to publish and disseminate information about the relevant invention, Technology and licensed patents, and to allow other nonprofit institutions to use, publish, or disseminate information about the relevant invention, Technology and licensed patents for educational and research purposes.

Under the UCSD Third License Agreement, we are obligated to achieve certain development milestones within specified time frames, to use commercially reasonable efforts to diligently develop, manufacture, and sell licensed products, and to meet agreed minimum-spend requirements. If we fail to perform any of these

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obligations, UCSD could either terminate the UCSD Third License Agreement or convert the exclusive license to a nonexclusive license subject to certain rights for us to extend these time frames.

The UCSD Third License Agreement required us to pay a one-time up-front non-refundable cash fee of \$20,000. We are obligated to pay annual license maintenance fees in the mid-four digits to low-five digits, increasing until such time that we are commercially selling a Product. We are also obligated to pay up to \$4.0 million upon the achievement of specified development and commercialization milestones for the first Product and low-to mid-single digit royalties based on aggregate net sales, subject to certain reductions for third-party licenses. The royalty term continues until the expiration of the UCSD Third License Agreement. If the royalties are below certain agreed amounts, we are required to pay UCSD minimum annual royalties ranging from low-to mid-five digits. We are also obligated to pay UCSD a percentage of sublicensing fees ranging in the low double digits.

The UCSD Third License Agreement will expire, unless earlier terminated, on the expiration of the last claim of any licensed patent or patent applications in a particular country. We have the right to terminate the UCSD Third License Agreement at any time upon sixty days' written notice to the UCSD. UCSD may terminate the UCSD Third License Agreement if we commit a material breach, if we fail to meet certain specified milestones within the prescribed time periods, if we are delinquent on any report or payment, if we are not diligently developing or commercializing Products in accordance with our diligence obligations, if we provide any intentionally false report, or if we challenge the validity of a licensed patent, in each case only if we fail to cure such problem within a specified cure period after written notice. In the event that we assign the UCSD Third License Agreement or we undergo a change of control, we will be obligated to pay a flat low-six digit fee or a fee that will be determined based on the acquisition price. Upon termination of the UCSD Third License Agreement, all licenses and rights granted by UCSD to us will terminate.

Sponsored research agreements with the Regents of University of California, San Diego

In connection with the UCSD Agreements, on December 3, 2021, we entered into a sponsored research agreement with the Regents of UCSD for our LX2020 and LX2021 programs, which was subsequently amended on April 5, 2023, and another sponsored research agreement with the Regents of UCSD for our LX2022 program, which was subsequently amended on April 19, 2023 and August 31, 2023. We refer to these agreements, as amended, as the Amended UCSD SRAs. UCSD reserved the rights to publish and disseminate information about the results generated from UCSD's investigator's conduct of the research. The total costs to be invoiced to us under the terms of the Amended UCSD SRAs are approximately \$5.0 million. Under the terms of the Amended UCSD SRAs, we have the first rights to obtain non-exclusive or exclusive, sublicensable, royalty-bearing, perpetual and transferable worldwide licenses to any inventions generated by UCSD or UCSD's interest in any inventions generated jointly by UCSD and us, and we retain the rights to any inventions generated solely by us.

Government regulation

Government authorities in the United States at federal, state and local levels, as well as in foreign countries, extensively regulate, among other things, the research, development, testing, manufacture, quality control, import, export, safety, effectiveness, labeling, packaging, storage, distribution, record keeping, approval, advertising, promotion, marketing, post-approval monitoring and post-approval reporting of biologics, including gene therapies, such as those we are developing. Generally, before a new biologic can be marketed, considerable data demonstrating its quality, safety and efficacy must be obtained, organized into a format specific for each regulatory authority, submitted for review and approved, authorized, or cleared by the applicable regulatory authority. We, along with third-party contractors, will be required to navigate the various preclinical, clinical and commercial approval requirements of the governing regulatory agencies of the countries in which we wish to conduct studies or seek approval or licensure of our product candidates.

U.S. biologics regulation

In the United States, biological products are subject to regulation under the U.S. Federal Food, Drug, and Cosmetic Act, or FDCA, and the Public Health Service Act, or PHSA, and their implementing regulations and other federal, state, local and foreign statutes and regulations. Failure to comply with the applicable United States requirements at any time during the product development process, approval process or following approval may subject an applicant to administrative actions or judicial sanctions. These actions and sanctions could include, among other actions, the FDA's refusal to approve pending applications, withdrawal of an approval, license revocation, a clinical hold, untitled or warning letters, voluntary or mandatory product recalls or market withdrawals, product seizures, total or partial suspension of production or distribution, injunctions, fines, refusals of government contracts, restitution, disgorgement and civil or criminal fines or penalties.

Our product candidates must be approved by the FDA through the Biologics License Application, or BLA, the process which is required by the FDA before biological product candidates may be marketed in the United States and generally involves the following:

- completion of extensive preclinical laboratory tests and animal studies performed in accordance with applicable regulations, including the FDA's good laboratory practices, or GLPs, regulations;
- submission to the FDA of an IND application, which must become effective before human clinical trials may begin;
- approval by an Institutional Review Board, or IRB, or ethics committee at each clinical site before the trial is commenced;
- performance of adequate and well-controlled human clinical trials in accordance with applicable IND regulations, the FDA's current Good Clinical Practices, or cGCPs, and other clinical trial-related regulations to establish the safety, purity and potency of the proposed biological product candidate for its intended purpose;
- preparation of and submission to the FDA of a BLA that contains sufficient data to demonstrate substantial evidence of effectiveness;
- a determination by the FDA within 60 days of its receipt of a BLA to file the application for review;
- payment of user fees for FDA review of the BLA;
- satisfactory completion of an FDA pre-license inspection of the manufacturing facility or facilities at which the proposed product will be produced to assess compliance with cGMPs and to assure that the facilities, methods and controls are adequate to ensure and preserve the biological product's identity, strength, quality and purity, and of selected clinical investigation sites to assess compliance with the cGCPs;
- satisfactory completion of an FDA Advisory Committee review, if applicable; and
- FDA review and approval, or licensure, of a BLA to permit commercial marketing of the product for particular indications for use in the United States.

Preclinical and clinical development

Prior to beginning the first clinical trial with a product candidate, the product candidate must undergo rigorous preclinical testing. Preclinical studies include laboratory evaluation of product chemistry and formulation, as well as *in vitro* and animal studies to assess safety and in some cases to establish a rationale for therapeutic use. The conduct of preclinical studies is subject to federal and state regulations and requirements, including GLP regulations for safety/toxicology studies.

An IND sponsor must submit the results of the preclinical tests, together with manufacturing information, analytical data, any available clinical data or literature and plans for clinical trials, among other things, to the

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FDA as part of an IND application to the FDA. An IND application is a request for authorization from the FDA to administer an IND product to humans. The central focus of an IND submission is on the general investigational plan and the protocol or protocols for preclinical studies and clinical trials. The IND application also includes results of animal and *in vitro* studies assessing the toxicology, pharmacokinetics, pharmacology and pharmacodynamic characteristics of the product, chemistry, manufacturing and controls information, and any available human data or literature to support the use of the investigational product. An IND application must become effective before human clinical trials may begin. The IND application automatically becomes effective 30 days after receipt by the FDA, unless the FDA, within the 30-day period, raises concerns or questions about the proposed clinical trial. In such a case, the IND application may be placed on clinical hold and the IND sponsor and the FDA must resolve any outstanding concerns or questions before the clinical trial can begin. Submission of an IND application therefore may or may not result in FDA authorization to begin a clinical trial. Additionally, the review of information in an IND submission may prompt FDA to, among other things, scrutinize existing INDs and could generate requests for information or clinical holds on other product candidates or programs.

Clinical trials involve the administration of the investigational product to human subjects under the supervision of qualified investigators, generally physicians not employed by or under the sponsor's control, in accordance with cGCPs, which include the requirement that all research subjects provide their informed consent for their participation in any clinical study. Clinical trials are conducted under protocols detailing, among other things, the objectives of the trial, dosing procedures, subject selection and exclusion criteria and the parameters to be used in monitoring safety and the effectiveness criteria to be evaluated. A separate submission to the existing IND application must be made for each successive clinical protocol conducted during product development and for any subsequent protocol amendments. Furthermore, an independent IRB for each site proposing to conduct the clinical trial must review and approve the plan for any clinical trial and its informed consent form before the clinical trial begins at that site, and must monitor the study until completed to ensure that the risks to individuals participating in the clinical trial are minimized and are reasonable in relation to anticipated benefits.

A sponsor who wishes to conduct a clinical trial outside of the United States may, but need not, obtain FDA authorization to conduct the clinical trial under an IND application. If a foreign clinical trial is not conducted under an IND application, the sponsor may submit data from the clinical trial to the FDA in support of a BLA. The FDA will accept a well-designed and well-conducted foreign clinical trial not conducted under an IND application if the foreign data are applicable to the United States population and medical practice, the trial was performed by clinical investigators of recognized competence, the trial was conducted in accordance with cGCP requirements, and the data may be considered valid without the need for an on-site inspection by the FDA or the FDA is able to validate the data through an onsite inspection if deemed necessary.

For purposes of BLA approval of a product candidate, human clinical trials are typically conducted in three sequential phases that may overlap or be combined:

- *Phase 1.* For gene therapies in general, the investigational product is initially introduced into patients with the target disease or condition. These studies are designed to test the safety, dosage tolerance, absorption, metabolism and distribution of the investigational product in humans, the side effects associated with increasing doses, and, if possible, to gain early evidence on effectiveness.
- *Phase 2.* The investigational product is administered to a limited patient population to evaluate the preliminary efficacy, optimal dosages and dosing schedule and to identify possible adverse side effects and safety risks.
- *Phase 3.* The investigational product is administered to an expanded patient population to further evaluate dosage, to provide statistically significant evidence of clinical efficacy and to further test for safety, generally at multiple geographically dispersed clinical trial sites. These clinical trials are intended to establish the overall risk/benefit ratio of the investigational product and to provide an adequate basis for product approval.

When these phases overlap or are combined, the trials may be referred to as Phase 1/2 or Phase 2/3.

In some cases, the FDA may require, or companies may voluntarily pursue, additional clinical trials after a product is approved to gain more information about the product. These so-called Phase 4 studies may be made a condition to approval of the BLA. Concurrent with clinical trials, companies may complete additional animal studies and develop additional information about the characteristics of the product candidate, and must finalize a process for manufacturing the product in commercial quantities in accordance with cGMP requirements. The manufacturing process must be capable of consistently producing quality batches of the product candidate and, among other things, must develop methods for testing the identity, strength, potency, quality and purity of the final product. Additionally, appropriate packaging must be selected and tested and stability studies must be conducted to demonstrate that the product candidate does not undergo unacceptable deterioration over its shelf life.

During all phases of clinical development, regulatory agencies require extensive monitoring and auditing of all clinical activities, clinical data and clinical study investigators. Written IND safety reports must be promptly submitted to the FDA and the investigators for serious, unexpected and suspected adverse events, any findings from other studies, tests in laboratory animals or in vitro testing that suggest a significant risk for human subjects, or any clinically important increase in the rate of a serious, and unexpected suspected adverse reaction over that listed in the protocol or investigator brochure. The sponsor must submit an IND safety report within 15 calendar days after the sponsor determines that the information qualifies for reporting. The sponsor also must notify the FDA of any unexpected fatal or life-threatening suspected adverse reaction within seven calendar days after the sponsor's initial receipt of the information. The FDA or the sponsor may suspend a clinical study at any time on various grounds, including a finding that the research patients or patients are being exposed to an unacceptable health risk. Similarly, an IRB can suspend or terminate approval of a clinical study at its institution if the clinical study is not being conducted in accordance with the IRB's requirements or if the biological product candidate has been associated with unexpected serious harm to patients. Some studies also include oversight by an independent group of qualified experts organized by the clinical study sponsor, known as a data safety monitoring board, which provides authorization for whether or not a study may move forward at designated checkpoints based on access to certain data from the study and may recommend halting the clinical trial if it determines that there is an unacceptable safety risk for subjects or other grounds, such as no demonstration of efficacy. There are also requirements governing the reporting of ongoing clinical trials and completed clinical trial results to public registries. Sponsors of clinical trials of FDA-regulated products, including biologics, are required to register and disclose certain clinical trial information, which is publicly available at www.clinicaltrials.gov.

BLA submission and review

Assuming successful completion of all required testing in accordance with all applicable regulatory requirements, the results of product development, preclinical studies and clinical trials are submitted to the FDA as part of a BLA requesting approval to market the product for one or more indications. FDA approval of a BLA must be obtained before a biologic may be marketed in the United States. The BLA must include all relevant data available from pertinent preclinical studies and clinical trials, including negative or ambiguous results as well as positive findings, together with detailed information relating to the product's chemistry, manufacturing, controls and proposed labeling, among other things. The submission of a BLA requires payment of a substantial application user fee to the FDA, unless a waiver or exemption applies. No user fees are assessed on BLAs for products designated as orphan drugs, unless the product also includes a non-orphan indication. The FDA reviews all submitted BLAs before it accepts them for filing, and may request additional information rather than accepting the BLA for filing. The FDA has sixty days from the applicant's submission of a BLA to either issue a refusal to file letter or accept the BLA for filing, indicating that it is sufficiently complete to permit substantive review.

Once a BLA has been accepted for filing, the FDA's goal is to review standard applications within ten months after it accepts the application for filing, or, if the application qualifies for priority review, six months

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after the FDA accepts the application for filing. In both standard and priority reviews, the review process can be significantly extended by FDA requests for additional information or clarification. The FDA reviews a BLA to determine, among other things, whether a product is safe, pure and potent for its intended use, and whether the facility in which it is manufactured, processed, packed or held meets standards designed to ensure and preserve the product's identity, safety, strength, quality, potency and purity. The FDA may convene an advisory committee, typically a panel that includes clinicians and other experts, to provide clinical insight on applications for novel products or products which present difficult questions of safety or efficacy. The advisory committee will provide a recommendation as to whether the application should be approved and under what conditions, if any. The FDA is not bound by the recommendations of an advisory committee, but it considers such recommendations when making decisions on approval. Before approving a BLA, the FDA will typically inspect the facility or facilities where the product is manufactured. The FDA will not approve an application unless it determines that the manufacturing processes and facilities are in compliance with cGMP requirements and adequate to assure consistent production of the product within required specifications. Additionally, before approving a BLA, the FDA will typically inspect one or more clinical sites to assure compliance with cGCPs. If the FDA determines that the application, manufacturing process or manufacturing facilities are not acceptable, it will outline the deficiencies in the submission and often will request additional testing or information. Notwithstanding the submission of any requested additional information, the FDA ultimately may decide that the application does not satisfy the regulatory criteria for approval.

After the FDA evaluates a BLA and conducts inspections of manufacturing facilities where the investigational product and/or its drug substance will be manufactured, the FDA may issue an approval letter or a Complete Response Letter. An approval letter authorizes commercial marketing of the product with specific prescribing information for specific indications. A Complete Response Letter indicates that the review cycle of the application is complete, and the application will not be approved in its present form. A Complete Response Letter will usually describe all of the deficiencies that the FDA has identified in the BLA, except that where the FDA determines that the data supporting the application are inadequate to support approval, the FDA may issue the Complete Response Letter without first conducting required inspections, testing submitted product lots and/or reviewing proposed labeling. In issuing the Complete Response Letter, the FDA may recommend actions that the applicant might take to place the BLA in condition for approval, including requests for additional information or clarification, which may include the potential requirement for additional preclinical studies or clinical trials or additional manufacturing activities. If a Complete Response Letter is issued, the applicant may either resubmit the BLA, addressing all of the deficiencies identified in the letter, or withdraw the application or request an opportunity for a hearing. The FDA may delay or refuse approval of a BLA if applicable regulatory criteria are not satisfied, require additional testing or information and/or require post-marketing testing and surveillance to monitor the safety or efficacy of a product.

If regulatory approval of a product is granted, such approval will be granted for particular indications and may entail limitations on the indicated uses for which such product may be marketed. For example, the FDA may approve the BLA with a Risk Evaluation and Mitigation Strategy, or REMS, to ensure the benefits of the product outweigh its risks. A REMS is a safety strategy to manage a known or potential serious risk associated with a product and to enable patients to have continued access to such medicines by managing their safe use, and could include medication guides, physician communication plans, or elements to assure safe use, such as restricted distribution methods, patient registries and other risk minimization tools. The FDA also may condition approval on, among other things, changes to proposed labeling or the development of adequate controls and specifications. Once approved, the FDA may withdraw the product approval if compliance with pre-and post-marketing requirements is not maintained or if problems occur after the product reaches the marketplace. The FDA may require one or more Phase 4 post-market studies and surveillance to further assess and monitor the product's safety and effectiveness after commercialization, and may limit further marketing of the product based on the results of these post-marketing studies.

Expedited development and review programs

The FDA offers a number of expedited development and reviews programs for qualifying product candidates. The Fast Track program is intended to expedite or facilitate the process for developing new products that meet certain criteria. Specifically, product candidates are eligible for Fast Track designation if they are intended to treat a serious or life-threatening disease or condition and demonstrate the potential to address unmet medical needs for the disease or condition. Fast Track designation applies to the combination of the product candidate and the specific indication for which it is being studied. The sponsor of a Fast Track designated product candidate has opportunities for frequent interactions with the FDA review team during product development and, once a BLA is submitted, the product candidate may be eligible for priority review. A Fast Track designated product candidate may also be eligible for rolling review, where the FDA may consider for review sections of the BLA on a rolling basis before the complete application is submitted, if the sponsor provides a schedule for the submission of the sections of the BLA, the FDA agrees to accept sections of the BLA and determines that the schedule is acceptable, and the sponsor pays any required user fees upon submission of the first section of the BLA. The sponsor can request the FDA to designate the product candidate for Fast Track status any time before receiving BLA approval, but ideally no later than the pre-BLA meeting.

A product candidate intended to treat a serious or life-threatening disease or condition may also be eligible for Breakthrough Therapy designation to expedite its development and review. A product candidate may receive Breakthrough Therapy designation if preliminary clinical evidence indicates that the product candidate, alone or in combination with one or more other drugs or biologics, may demonstrate substantial improvement over available therapies on one or more clinically significant endpoints, such as substantial treatment effects observed early in clinical development. The designation includes all of the Fast Track program features, as well as more intensive FDA interaction and guidance beginning as early as Phase 1 and an organizational commitment to expedite the development and review of the product candidate, including involvement of senior managers.

As part of the 21st Century Cures Act, Congress amended the FDCA to facilitate an efficient development program for, and expedite review of regenerative medicine therapies, which include cell therapies, therapeutic tissue engineering products, human cell and tissue products, and combination products using any such therapies or products. Gene therapies, including genetically modified cells that lead to a durable modification of cells or tissues may meet the definition of a “regenerative medicine therapy.” A product candidate may be eligible for regenerative medicine therapy, or RMAT, designation if it meets the following criteria: (1) it is a regenerative medicine therapy; (2) it is intended to treat, modify, reverse, or cure a serious or life-threatening disease or condition; and (3) preliminary clinical evidence indicates that it has the potential to address unmet medical needs for such a disease or condition. A sponsor may request that the FDA designate a product candidate as an RMAT concurrently with or at any time after the submission of an IND. A BLA for a product candidate that has received RMAT designation may be eligible for priority review or accelerated approval through (1) surrogate or intermediate endpoints reasonably likely to predict long-term clinical benefit or (2) reliance upon data obtained from a meaningful number of sites. Benefits of such designation also include early interactions with FDA to discuss any potential surrogate or intermediate endpoint to be used to support accelerated approval. A product candidate with RMAT designation that is granted accelerated approval and is subject to post-approval requirements may fulfill such requirements through the submission of clinical evidence, clinical studies, patient registries, or other sources of real world evidence, such as electronic health records; the collection of larger confirmatory data sets; or post-approval monitoring of all patients treated with such therapy prior to its approval.

Any marketing application for a biologic submitted to the FDA for approval, including a product with a Fast Track designation and/or Breakthrough Therapy designation, may be eligible for other types of FDA programs intended to expedite the FDA review and approval process, such as priority review. A product is eligible for priority review if it is designed to treat a serious or life threatening disease or condition and, if approved, would provide a significant improvement in safety and effectiveness compared to available therapies. For original BLAs, priority review designation means the FDA’s goal is to take action on the marketing application within six months of the 60-day filing date.

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Additionally, a product candidate may be eligible for accelerated approval if it is designed to treat a serious or life-threatening disease or condition and demonstrates an effect on a surrogate endpoint that is reasonably likely to predict clinical benefit, or on a clinical endpoint that can be measured earlier than irreversible morbidity or mortality, or IMM, that is reasonably likely to predict an effect on IMM or other clinical benefit, taking into account the severity, rarity, or prevalence of the condition and the availability or lack of alternative treatments. As a condition of accelerated approval, the FDA will generally require the sponsor to perform adequate and well-controlled post-marketing clinical studies with due diligence to verify and describe the anticipated effect on IMM or other clinical benefit. The FDA may withdraw approval of a product or indication approved under accelerated approval if, for example, the confirmatory trial fails to verify the predicted clinical benefit of the product. In addition, for products being considered for accelerated approval, the FDA generally requires, unless otherwise informed by the agency, pre-approval of all advertising and promotional materials intended for dissemination or publication, which could adversely impact the timing of the commercial launch of the product. The Food and Drug Omnibus Reform Act also made several changes to the FDA's authorities and its regulatory framework, including, among other changes, reforms to the accelerated approval pathway, such as requiring the FDA to specify conditions for post-approval study requirements and setting forth procedures for the FDA to withdraw a product on an expedited basis for non-compliance with post-approval requirements.

Fast Track designation, Breakthrough Therapy designation, RMAT designation, and priority review do not change the standards for approval but may expedite the development or approval process. Even if a product qualifies for one or more of these programs, the FDA may later decide that the product no longer meets the conditions for qualification or decide that the time period for FDA review or approval will not be shortened.

Orphan Drug designation

Under the Orphan Drug Act, the FDA may grant Orphan Drug designation to a product candidate intended to treat a rare disease or condition, which is generally a disease or condition that affects fewer than 200,000 individuals in the United States, or 200,000 or more individuals in the United States for which there is no reasonable expectation that the cost of developing and making available in the United States a drug or biologic for this type of disease or condition will be recovered from sales in the United States for that product candidate. Orphan Drug designation must be requested before submitting a BLA. After the FDA grants Orphan Drug designation, the identity of the therapeutic agent and its potential orphan use are disclosed publicly by the FDA. The Orphan Drug designation does not convey any advantage in, or shorten the duration of, the regulatory review or approval process.

If a product that has Orphan Drug designation subsequently receives the first FDA approval for the disease or condition for which it has such designation, the product is entitled to orphan drug exclusive approval (or exclusivity), which means that the FDA may not approve any other applications, including a full BLA, to market the same product for the same indication for seven years, except in limited circumstances, such as a showing of clinical superiority to the product with orphan drug exclusivity by means of greater effectiveness, greater safety or providing a major contribution to patient care or if the holder of the orphan drug exclusivity cannot assure the availability of sufficient quantities of the orphan drug to meet the needs of patients with the disease or condition for which the product was designated. Orphan drug exclusivity does not prevent the FDA from approving a different drug or biologic for the same disease or condition, or the same drug or biologic for a different disease or condition. Among the other benefits of Orphan Drug designation are tax credits for certain research and a waiver of the BLA application fee.

A designated orphan drug may not receive orphan drug exclusivity if it is approved for a use that is broader than the indication for which it received Orphan Drug designation. In addition, exclusive marketing rights in the United States may be lost if the FDA later determines that the request for designation was materially defective or if the manufacturer is unable to assure sufficient quantities of the product to meet the needs of patients with the rare disease or condition. In view of the court decision in *Catalyst Pharms., Inc. v. Becerra*, 14 F.4th 1299 (11th Cir. 2021), in January 2023, the FDA published a notice in the Federal Register to clarify that while the agency

complies with the court's order in *Catalyst*, FDA intends to continue to apply its longstanding interpretation of the regulations to matters outside of the scope of the *Catalyst* order – that is, the agency will continue tying the scope of orphan-drug exclusivity to the uses or indications for which a drug is approved, which permits other sponsors to obtain approval of a drug for new uses or indications within the same orphan designated disease or condition that have not yet been approved. It is unclear how future litigation, legislation, agency decisions, and administrative actions will impact the scope of the orphan drug exclusivity.

Rare Pediatric Disease designation and priority review vouchers

Under the FDCA, as amended, the FDA incentivizes the development of product candidates that meet the definition of a “rare pediatric disease,” defined to mean a serious or life-threatening disease in which the serious or life-threatening manifestations primarily affect individuals aged from birth to 18 years and the disease affects fewer than 200,000 individuals in the United States or affects 200,000 or more in the United States and for which there is no reasonable expectation that the cost of developing and making in the United States a drug for such disease or condition will be received from sales in the United States of such drug. The sponsor of a product candidate for a rare pediatric disease may be eligible for a voucher that can be used to obtain a priority review for a subsequent human drug or biologic application after the date of approval of the rare pediatric disease drug product. A sponsor may request Rare Pediatric Disease designation from the FDA prior to the submission of its BLA. A Rare Pediatric Disease designation does not guarantee that a sponsor will receive a priority review voucher, or PRV, upon approval of its BLA. Moreover, a sponsor who chooses not to submit a Rare Pediatric Disease designation request may nonetheless receive a PRV upon approval of their marketing application if they request such a voucher in their original marketing application and meet all of the eligibility criteria. If a PRV is received, it may be sold or transferred an unlimited number of times. Congress has extended the PRV program until September 30, 2024, with the potential for PRVs to be granted until September 30, 2026.

Pediatric information and pediatric exclusivity

Under the Pediatric Research Equity Act, or PREA, certain BLAs and certain supplements to a BLA must contain data to assess the safety and efficacy of the product for the claimed indications in all relevant pediatric subpopulations and to support dosing and administration for each pediatric subpopulation for which the product is safe and effective. The FDA may grant deferrals for the submission of pediatric data or full or partial waivers. The Food and Drug Administration Safety and Innovation Act, or FDASIA, amended the FDCA to require that a sponsor who is planning to submit a marketing application for a product that includes a new active ingredient, new indication, new dosage form, new dosing regimen or new route of administration submit an initial Pediatric Study Plan, or PSP, within 60 days of an end-of-Phase 2 meeting or, if there is no such meeting, as early as practicable before the initiation of the Phase 3 or Phase 2/3 study. The initial PSP must include an outline of the pediatric study or studies that the sponsor plans to conduct, including study objectives and design, age groups, relevant endpoints and statistical approach, or a justification for not including such detailed information, and any request for a deferral of pediatric assessments or a full or partial waiver of the requirement to provide data from pediatric studies along with supporting information. The FDA and the sponsor must reach an agreement on the PSP. A sponsor can submit amendments to an agreed-upon initial PSP at any time if changes to the pediatric plan need to be considered based on data collected from preclinical studies, early phase clinical trials and/or other clinical development programs. A biological product can also obtain pediatric market exclusivity in the United States. Pediatric exclusivity, if granted, adds six months to existing exclusivity periods. This six-month exclusivity, which runs from the end of other exclusivity protection, may be granted based on the voluntary completion of a pediatric study in accordance with an FDA-issued “Written Request” for such a study.

Post-approval requirements

Any products manufactured or distributed by us pursuant to FDA approvals are subject to pervasive and continuing regulation by the FDA, including, among other things, requirements relating to record-keeping, reporting of adverse experiences, periodic reporting, product sampling and distribution, and advertising and

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promotion of the product. As part of the manufacturing process, the manufacturer is required to perform certain tests on each lot of the product before it is released for distribution. After a BLA is approved for a biological product, the product also may be subject to official lot release. If the product is subject to official release by the FDA, the manufacturer submits samples of each lot of product to the FDA together with a release protocol showing a summary of the history of manufacture of the lot and the results of all of the manufacturer's tests performed on the lot. The FDA also may perform certain confirmatory tests on lots of some products before releasing the lots for distribution by the manufacturer. In addition, the FDA conducts laboratory research related to the regulatory standards on the safety, purity, potency and effectiveness of biologics. After approval, most changes to the approved product, such as adding new indications or other labeling claims, are subject to prior FDA review and approval. There also are continuing user fee requirements, under which the FDA assesses an annual program fee for each product identified in an approved BLA. Biologic manufacturers and their subcontractors are required to register their establishments with the FDA and certain state agencies, and are subject to periodic unannounced inspections by the FDA and certain state agencies for compliance with cGMPs, which impose certain procedural and documentation requirements upon us and our third-party manufacturers. Changes to the manufacturing process are strictly regulated, and, depending on the significance of the change, may require prior FDA approval before being implemented. FDA regulations also require investigation and correction of any deviations from cGMPs and impose reporting requirements upon us and any third-party manufacturers that we may decide to use. Manufacturers and other parties involved in the drug supply chain for prescription drug products must also comply with product tracking and tracing requirements and for notifying the FDA of counterfeit, diverted, stolen and intentionally adulterated products or products that are otherwise unfit for distribution in the United States. Accordingly, manufacturers must continue to expend time, money and effort in the area of production and quality control to maintain compliance with cGMPs and other aspects of regulatory compliance.

The FDA may withdraw approval if compliance with regulatory requirements and standards is not maintained or if problems occur after the product reaches the market. Later discovery of previously unknown problems with a product, including adverse events of unanticipated severity or frequency, or with manufacturing processes, or failure to comply with regulatory requirements, may result in revisions to the approved labeling to add new safety information; imposition of post-market studies or clinical studies to assess new safety risks; or imposition of distribution restrictions or other restrictions under a REMS program. Other potential consequences include, among other things:

- restrictions on the marketing or manufacturing of a product, complete withdrawal of the product from the market or product recalls;
- fines, warning or untitled letters or holds on post-approval clinical studies;
- refusal of the FDA to approve pending applications or supplements to approved applications, or suspension or revocation of existing product approvals;
- product seizure or detention, or refusal of the FDA to permit the import or export of products;
- consent decrees, corporate integrity agreements, debarment or exclusion from federal healthcare programs;
- mandated modification of promotional materials and labeling and the issuance of corrective information;
- the issuance of safety alerts, Dear Healthcare Provider letters, press releases and other communications containing warnings or other safety information about the product; or
- injunctions or the imposition of civil or criminal penalties.

The FDA closely regulates the marketing, labeling, advertising and promotion of biologics. A company can make only those claims relating to safety and efficacy, purity and potency that are approved by the FDA and in accordance with the provisions of the approved label. However, companies may share truthful and not misleading

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information that is otherwise consistent with a product's FDA-approved labeling. The FDA and other agencies actively enforce the laws and regulations prohibiting the promotion of off-label uses. Failure to comply with these requirements can result in, among other things, adverse publicity, warning letters, corrective advertising and potential civil and criminal penalties. Physicians may prescribe legally available products for uses that are not described in the product's labeling and that differ from those tested by us and approved by the FDA. Such off-label uses are common across medical specialties. Physicians may believe that such off-label uses are the best treatment for many patients in varied circumstances. The FDA does not regulate the behavior of physicians in their choice of treatments. The FDA does, however, restrict manufacturer's communications on the subject of off-label use of their products.

Biosimilars and reference product exclusivity

The Patient Protection and Affordable Care Act, as amended by the Health Care and Education Reconciliation Act, or collectively, the ACA, includes a subtitle called the Biologics Price Competition and Innovation Act, or BPCIA, which created an abbreviated approval pathway for biologics that are biosimilar to or interchangeable with an FDA-approved reference biological product. To date, a number of biosimilars have been licensed under the BPCIA, and numerous biosimilars have been approved in Europe. The FDA has issued several guidance documents outlining an approach to review and approval of biosimilars.

Biosimilarity, which requires that there be no clinically meaningful differences between the biological product and the reference product in terms of safety, purity and potency, can be shown through analytical studies, animal studies and a clinical study or studies. Interchangeability requires that a product is biosimilar to the reference product and the product must demonstrate that it can be expected to produce the same clinical results as the reference product in any given patient and, for products that are administered multiple times to an individual, the biologic and the reference biologic may be alternated or switched after one has been previously administered without increasing safety risks or risks of diminished efficacy relative to exclusive use of the reference biologic. Complexities associated with the larger, and often more complex, structures of biologics, as well as the processes by which such products are manufactured, pose significant hurdles to the implementation of the abbreviated approval pathway that are still being worked out by the FDA.

Under the BPCIA, an application for a biosimilar product may not be submitted to the FDA until four years following the date that the reference product was first licensed by the FDA. In addition, the approval of a biosimilar product may not be made effective by the FDA until 12 years from the date on which the reference product was first licensed. During this 12-year period of exclusivity, another company may still market a competing version of the reference product if the FDA approves a full BLA for the competing product containing that applicant's own preclinical data and data from adequate and well-controlled clinical trials to demonstrate the safety, purity and potency of its product. The BPCIA also created certain exclusivity periods for biosimilars approved as interchangeable products. At this juncture, it is unclear whether products deemed "interchangeable" by the FDA will, in fact, be readily substituted by pharmacies, which are governed by state pharmacy law.

The BPCIA is complex and continues to be interpreted and implemented by the FDA. In addition, government proposals have sought to reduce the 12-year reference product exclusivity period. Other aspects of the BPCIA, some of which may impact the BPCIA exclusivity provisions, have also been the subject of recent litigation. As a result, the ultimate impact and implementation of the BPCIA are subject to significant uncertainty.

Regulation of companion diagnostics

If an in vitro diagnostic, which is regulated by the FDA as a medical device, is essential to the safe and effective use of a therapeutic and is not available on the market, then the FDA generally will require approval or clearance of that diagnostic, known as a companion diagnostic, at the same time that the FDA approves the therapeutic product. In August 2014, the FDA issued final guidance clarifying the requirements that will apply to

approval of therapeutic products and *in vitro* companion diagnostics. According to the guidance, an unapproved or uncleared companion diagnostic device used to make treatment decisions in clinical trials of a drug generally will be considered an investigational medical device unless it is employed for an intended use for which the device is already approved or cleared. If used to make critical treatment decisions, such as patient selection, the diagnostic device generally will be considered a significant risk device under the FDA's Investigational Device Exemption, or IDE, regulations. The sponsor of the diagnostic device will be required to comply with the IDE regulations for clinical studies involving the investigational diagnostic device. According to the guidance, if a diagnostic device and a drug are to be studied together to support their respective approvals, both products can be studied in the same clinical trial, if the trial meets both the requirements of the IDE regulations and the IND regulations. The guidance provides that depending on the details of the clinical trial protocol, the investigational product(s), and subjects involved, a sponsor may seek to submit an IDE alone (e.g., if the drug has already been approved by FDA and is used consistent with its approved labeling), or both an IND and an IDE.

Pursuing FDA approval/clearance of an *in vitro* companion diagnostic would require either a pre-market notification, also called 510(k) clearance, or a pre-market approval, or PMA, or a *de novo* classification for that diagnostic. The review of companion diagnostics involves coordination of review with the FDA's Center for Devices and Radiological Health. Once cleared or approved, the companion diagnostic must adhere to post-marketing requirements including the requirements of FDA's quality system regulation, medical device reporting, recalls and corrections along with product marketing requirements and limitations. Companion diagnostic manufacturers are subject to unannounced FDA inspections at any time during which the FDA will conduct an audit of the product(s) and the company's facilities for compliance with its authorities.

510(k) clearance process

To obtain 510(k) clearance, a pre-market notification is submitted to the FDA demonstrating that the proposed device is substantially equivalent to a previously cleared 510(k) device or a device that was in commercial distribution before May 28, 1976 for which the FDA has not yet required the submission of a PMA application. The FDA's 510(k) clearance process may take three to 12 months from the date the application is submitted and filed with the FDA, but may take longer if FDA requests additional information, among other reasons. In some cases, the FDA may require clinical data to support substantial equivalence. In reviewing a pre-market notification submission, the FDA may request additional information, which may significantly prolong the review process. Notwithstanding compliance with all these requirements, clearance is never assured. After a device receives 510(k) clearance, any subsequent modification of the device that could significantly affect its safety or effectiveness, or that would constitute a major change in its intended use, will require a new 510(k) clearance or require a PMA. In addition, the FDA may make substantial changes to industry requirements, including which devices are eligible for 510(k) clearance, which may significantly affect the process.

De novo classification process

If a new medical device does not qualify for the 510(k) pre-market notification process because no predicate device to which it is substantially equivalent can be identified, the device is automatically classified into Class III. The Food and Drug Administration Modernization Act of 1997 established a different route to market for low to moderate risk medical devices that are automatically placed into Class III due to the absence of a predicate device, called the "Request for Evaluation of Automatic Class III Designation," or the *de novo* classification process. This process allows a manufacturer whose novel device is automatically classified into Class III to request down-classification of its medical device into Class I or Class II on the basis that the device presents low or moderate risk, rather than requiring the submission and approval of a PMA. If the manufacturer seeks reclassification into Class II, the manufacturer must include a draft proposal for special controls that are necessary to provide a reasonable assurance of the safety and effectiveness of the medical device. The FDA may reject the reclassification petition if it identifies a legally marketed predicate device that would be appropriate for a 510(k) or determines that the device is not low to moderate risk and requires PMA or that general controls would be inadequate to control the risks and special controls cannot be developed. Obtaining FDA marketing

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authorization, de novo down-classification, or approval for medical devices is expensive and uncertain, and may take several years, and generally requires significant scientific and clinical data.

PMA process

The PMA process, including the gathering of clinical and nonclinical data and the submission to and review by the FDA, can take several years or longer. The applicant must prepare and provide the FDA with reasonable assurance of the device's safety and effectiveness, including information about the device and its components regarding, among other things, device design, manufacturing, and labeling. PMA applications are subject to an application fee. In addition, PMAs for medical devices must generally include the results from extensive preclinical and adequate and well-controlled clinical trials to establish the safety and effectiveness of the device for each indication for which FDA approval is sought. In particular, for a diagnostic, the applicant must demonstrate that the diagnostic produces reproducible results. As part of the PMA review, the FDA will typically inspect the manufacturer's facilities for compliance with the Quality System Regulation, which imposes extensive testing, control, documentation, and other quality assurance and GMP requirements.

Foreign regulation

In order to market any product outside of the United States, we would need to comply with numerous and varying regulatory requirements of other countries and jurisdictions regarding quality, safety, and efficacy and governing, among other things, clinical trials, marketing authorization, commercial sales and distribution of our products. Whether or not we obtain FDA approval for a product, we would need to obtain the necessary approvals by the comparable foreign regulatory authorities before we can commence clinical trials or marketing of the product in foreign countries and jurisdictions. Although many of the issues discussed above with respect to the United States apply similarly in the context of the European Union, the approval process varies between countries and jurisdictions and can involve additional product testing and additional administrative review periods. The time required to obtain approval in other countries and jurisdictions might differ from and be longer than that required to obtain FDA approval. Regulatory approval in one country or jurisdiction does not ensure regulatory approval in another, but a failure or delay in obtaining regulatory approval in one country or jurisdiction may negatively impact the regulatory process in others.

Other healthcare laws and compliance requirements

Pharmaceutical companies are subject to additional healthcare regulation and enforcement by the federal government and by authorities in the states and foreign jurisdictions in which they conduct their business, particularly once they have a commercialized product that is reimbursable by third-party payor programs. Such laws include, without limitation: the U.S. federal Anti-Kickback Statute, the civil False Claims Act, the federal Health Insurance Portability and Accountability Act of 1996, or HIPAA, and similar foreign, federal and state fraud and abuse, transparency and privacy laws.

- The U.S. federal Anti-Kickback Statute prohibits, among other things, persons and entities from knowingly and willfully soliciting, receiving, offering or paying remuneration, to induce, or in return for, either the referral of an individual, or the purchase or recommendation of an item or service for which payment may be made under any federal healthcare program. The term remuneration has been interpreted broadly to include anything of value, including stock options. There are a number of statutory exceptions and regulatory safe harbors protecting some common activities from prosecution, but they are drawn narrowly, and practices that involve remuneration, such as consulting agreements, that may be alleged to be intended to induce prescribing, purchasing or recommending may be subject to scrutiny if they do not qualify for an exception or safe harbor. Our practices may not in all cases meet all of the criteria for protection under a statutory exception or regulatory safe harbor. A person or entity does not need to have actual knowledge of the statute or specific intent to violate it in order to have committed a violation. Violations are subject to civil and criminal fines and penalties for each

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violation, plus up to three times the remuneration involved, imprisonment, and exclusion from government healthcare programs. In addition, a claim including items or services resulting from a violation of the U.S. federal Anti-Kickback Statute constitutes a false or fraudulent claim for purposes of the civil False Claims Act.

- Civil and criminal false claims laws, and civil monetary penalty laws, including the civil False Claims Act, which can be enforced through civil whistleblower or qui tam actions, prohibit, among other things, individuals or entities from knowingly presenting, or causing to be presented, claims for payment to the federal government, including federal healthcare programs, that are false or fraudulent. For example, the civil False Claims Act prohibits any person or entity from knowingly presenting, or causing to be presented, a false claim for payment to the federal government or knowingly making, using or causing to be made or used a false record or statement material to a false or fraudulent claim to the federal government.
- HIPAA created additional federal civil and criminal liability for, among other things, knowingly and willfully executing a scheme to defraud any healthcare benefit program, or obtain, by means of false or fraudulent pretenses, representations, or promises, any of the money or property owned by, or under the custody or control of, any healthcare benefit program, regardless of the payor (e.g., public or private) and knowingly and willfully falsifying, concealing, or covering up by any trick or device a material fact or making any materially false statements in connection with the delivery of, or payment for, healthcare benefits, items or services relating to healthcare matters. Similar to the U.S. federal Anti-Kickback Statute, a person or entity can be found guilty of violating HIPAA without actual knowledge of the statute or specific intent to violate it.
- HIPAA, as amended by the Health Information Technology for Economic and Clinical Health Act of 2009, or HITECH, and their respective implementing regulations, impose certain requirements on HIPAA covered entities, which include certain healthcare providers, healthcare clearing houses and health plans, and individuals and entities that provide services on their behalf that involve individually identifiable health information, known as business associates, relating to the privacy, security and transmission of individually identifiable health information, as well as their covered subcontractors. HITECH also created new tiers of civil monetary penalties, amended HIPAA to make civil and criminal penalties directly applicable to business associates, and gave state attorneys general new authority to file civil actions for damages or injunctions in federal courts to enforce the federal HIPAA laws and seek attorneys' fees and costs associated with pursuing federal civil actions.
- The U.S. federal Physician Payments Sunshine Act requires certain manufacturers of drugs, devices, biologics and medical supplies for which payment is available under Medicare, Medicaid or the Children's Health Insurance Program, with specific exceptions, to annually report to the Centers for Medicare and Medicaid Services, or CMS, information related to payments and other transfers of value made to physicians (defined to include doctors, dentists, optometrists, podiatrists and chiropractors), certain other licensed healthcare professionals (such as physician assistants and nurse practitioners), and teaching hospitals, as well as ownership and investment interests held by physicians and their immediate family members.

We may also be subject to additional similar U.S. state and foreign law equivalents of each of the above federal laws, such as anti-kickback and false claims laws which may apply to sales or marketing arrangements and claims involving healthcare items or services reimbursed by non-governmental third-party payors, including private insurers, or that apply regardless of payor, state laws which require pharmaceutical companies to comply with the pharmaceutical industry's voluntary compliance guidelines and the relevant compliance guidance promulgated by the federal government, state and local laws which require pharmaceutical companies to report information related to payments and other transfers of value to physicians and other healthcare providers or marketing expenditures, state laws which require the reporting of information related to product pricing, state and local laws requiring the registration of pharmaceutical sales representatives, and state and foreign laws governing the privacy and security of health information which, in some cases, differ from each other in significant ways,

and may not have the same effect, thus complicating compliance efforts. If our operations are found to be in violation of any of such laws or any other governmental regulations that apply, we may be subject to penalties, including, without limitation, significant civil, criminal and administrative penalties, damages, fines, exclusion from government-funded healthcare programs, such as Medicare and Medicaid or similar programs in other countries or jurisdictions, integrity oversight and reporting obligations to resolve allegations of non-compliance, disgorgement, imprisonment, contractual damages, reputational harm, diminished profits and the curtailment or restructuring of our operations.

Coverage and reimbursement

Significant uncertainty exists as to the coverage and reimbursement status of any pharmaceutical or biological product for which we obtain regulatory approval in the future. Sales of any product, if approved, depend, in part, on the extent to which such product will be covered by third-party payors, such as federal, state and foreign government healthcare programs, commercial insurance and managed healthcare organizations, and the level of reimbursement, if any, for such product by third-party payors. Decisions regarding whether to cover any of our product candidates, if approved, the extent of coverage and amount of reimbursement to be provided are made on a plan-by-plan basis. Further, no uniform policy for coverage and reimbursement exists in the United States, and coverage and reimbursement can differ significantly from payor to payor.

Third-party payors often rely upon Medicare coverage policy and payment limitations in setting their own reimbursement rates, but also have their own methods and approval process apart from Medicare determinations. As a result, the coverage determination process is often a time-consuming and costly process that will require us to provide scientific and clinical support for the use of our product candidates to each payor separately, with no assurance that coverage and adequate reimbursement will be applied consistently or obtained in the first instance. For example, the FDA recently approved a monoclonal antibody treatment for Alzheimer's disease, aducanumab (Aduhelm), which is the first Alzheimer's disease drug to be approved in nearly 20 years. On April 7, 2022, CMS released a national coverage determination for FDA-approved monoclonal antibodies directed against amyloid for the treatment of Alzheimer's disease, including aducanumab (Aduhelm). Further, on January 6, 2023, CMS announced that this coverage determination also applies to lecanemab (Leqembi). Nevertheless, it is possible that CMS may not approve Medicare coverage and reimbursement for any of our product candidates for treatment of Alzheimer's disease, once approved.

Furthermore, it is possible that one or more of our product candidates may not be considered medically necessary or cost effective. A decision by a third-party payor not to cover any product candidates we may develop could reduce physician utilization of such product candidates once approved and have a material adverse effect on our sales, results of operations and financial condition.

For products administered under the supervision of a physician, obtaining coverage and adequate reimbursement may be particularly difficult because of the higher prices often associated with such drugs. Additionally, separate reimbursement for the product itself or the treatment or procedure in which the product is used may not be available, which may impact physician utilization.

In addition, the U.S. government, state legislatures and foreign governments have continued implementing cost-containment programs, including price controls, restrictions on coverage and reimbursement and requirements for substitution of generic products. Third-party payors are increasingly challenging the prices charged for medical products and services, examining the medical necessity and reviewing the cost-effectiveness of pharmaceutical or biologics, medical devices and medical services, in addition to questioning safety and efficacy. Adoption of price controls and cost-containment measures, and adoption of more restrictive policies in jurisdictions with existing controls and measures, could further limit sales of any product that receives approval. Decreases in third-party reimbursement for any product or a decision by a third party not to cover a product could reduce physician usage and patient demand for the product. In addition, companion diagnostic tests require coverage and reimbursement separate and apart from the coverage and reimbursement for their companion

pharmaceutical or biological products. Similar challenges to obtaining coverage and reimbursement, applicable to pharmaceutical or biological products, will apply to companion diagnostics.

Outside the United States, ensuring adequate coverage and payment for any biological candidates we may develop will face challenges. Pricing of prescription pharmaceuticals is subject to governmental control in many countries. Pricing negotiations with governmental authorities can extend well beyond the receipt of regulatory marketing approval for a product and may require us to conduct a clinical trial that compares the cost effectiveness of any product candidates we may develop to other available therapies. The conduct of such a clinical trial could be expensive and result in delays in our commercialization efforts. In the European Union, pricing and reimbursement schemes vary widely from country to country. Some countries provide that products may be marketed only after a reimbursement price has been agreed. Some countries may require the completion of additional studies that compare the cost-effectiveness of a particular product candidate to currently available therapies (so called health technology assessments) in order to obtain reimbursement or pricing approval. For example, the European Union provides options for its member states to restrict the range of products for which their national health insurance systems provide reimbursement and to control the prices of medicinal products for human use. European Union member states may approve a specific price for a product or it may instead adopt a system of direct or indirect controls on the profitability of the company placing the product on the market. Other member states allow companies to fix their own prices for products but monitor and control prescription volumes and issue guidance to physicians to limit prescriptions. Recently, many countries in the European Union have increased the amount of discounts required on pharmaceuticals and these efforts could continue as countries attempt to manage healthcare expenditures, especially in light of the severe fiscal and debt crises experienced by many countries in the European Union. The downward pressure on healthcare costs in general, particularly prescription products, has become intense. As a result, increasingly high barriers are being erected to the entry of new products. Political, economic and regulatory developments may further complicate pricing negotiations, and pricing negotiations may continue after reimbursement has been obtained. Reference pricing used by various European Union member states, and parallel trade (arbitrage between low-priced and high-priced member states), can further reduce prices. There can be no assurance that any country that has price controls or reimbursement limitations for pharmaceutical products will allow favorable reimbursement and pricing arrangements for any of our products, if approved in those countries.

Healthcare reform

The United States and some foreign jurisdictions are considering or have enacted a number of reform proposals to change the healthcare system. There is significant interest in promoting changes in healthcare systems with the stated goals of containing healthcare costs, improving quality or expanding access. In the United States, the pharmaceutical industry has been a particular focus of these efforts and has been significantly affected by federal and state legislative initiatives, including those designed to limit the pricing, coverage and reimbursement of pharmaceutical and biopharmaceutical products, especially under government-funded healthcare programs, and increased governmental control of drug pricing.

The ACA, which was enacted in 2010, substantially changed the way healthcare is financed by both governmental and private insurers in the United States, and significantly affected the pharmaceutical industry. In addition, other legislative and regulatory changes have been proposed and adopted in the United States since the ACA was enacted:

- The Budget Control Act of 2011 and subsequent legislation, among other things, created measures for spending reductions by Congress that include aggregate reductions of Medicare payments to providers of 2% per fiscal year, which remain in effect through 2032. Due to the Statutory Pay-As-You-Go Act of 2010, estimated budget deficit increases resulting from the American Rescue Plan Act of 2021, and subsequent legislation, Medicare payments to providers will be further reduced starting in 2025 absent further legislation. The U.S. American Taxpayer Relief Act of 2012 further reduced Medicare payments to several types of providers and increased the statute of limitations period for the government to recover overpayments to providers from three to five years.

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- On April 13, 2017, CMS published a final rule that gives states greater flexibility in setting benchmarks for insurers in the individual and small group marketplaces, which may have the effect of relaxing the essential health benefits required under the ACA for plans sold through such marketplaces.
- On May 23, 2019, CMS published a final rule to allow Medicare Advantage Plans the option of using step therapy for Part B drugs beginning January 1, 2020.

Additionally, there has been heightened governmental scrutiny over the manner in which manufacturers set prices for their marketed products, which has resulted in several U.S. presidential executive orders, Congressional inquiries and proposed and enacted federal legislation designed to, among other things, bring more transparency to drug pricing, reduce the cost of prescription drugs under Medicare, and review the relationship between pricing and manufacturer patient programs. The Inflation Reduction Act of 2022, or the IRA, includes several provisions that may impact our business to varying degrees, including provisions that reduce the out-of-pocket spending cap for Medicare Part D beneficiaries from \$7,050 to \$2,000 starting in 2025, thereby effectively eliminating the coverage gap; impose new manufacturer financial liability on certain drugs under Medicare Part D, allow the U.S. government to negotiate Medicare Part B and Part D price caps for certain high-cost drugs and biologics without generic or biosimilar competition; require companies to pay rebates to Medicare for certain drug prices that increase faster than inflation; and delay until January 1, 2032 the implementation of the U.S. Department of Health and Human Services, or the HHS, rebate rule that would have limited the fees that pharmacy benefit managers can charge. The IRA permits HHS to implement many of these provisions through guidance, as opposed to regulation, for the initial years. These provisions took effect progressively starting in fiscal year 2023, although they may be subject to legal challenges. HHS has and will continue to issue and update guidance as these programs are implemented. Further, under the IRA, orphan drugs are exempted from the Medicare drug price negotiation program, but only if they have one rare disease designation and for which the only approved indication is for that disease or condition. If a product receives multiple rare disease designations or has multiple approved indications, it may not qualify for the orphan drug exemption. Various industry stakeholders, including certain pharmaceutical companies and the Pharmaceutical Research and Manufacturers of America, have initiated lawsuits against the federal government asserting that the price negotiation provisions of IRA are unconstitutional. It is currently unclear how these judicial challenges as well as other legislative, executive, and administrative actions, including how the IRA will be implemented, will impact the pharmaceutical industry. The implementation of cost containment measures or other healthcare reforms may prevent us from being able to generate revenue, attain profitability, or commercialize our product candidates if approved.

In addition, in response to the Biden administration's October 2022 executive order, on February 14, 2023, HHS released a report outlining three new models for testing by the Center for Medicare and Medicaid Innovation which will be evaluated on their ability to lower the cost of drugs, promote accessibility, and improve quality of care. It is unclear whether the models will be utilized in any health reform measures in the future. Although a number of these and other proposed measures may require authorization through additional legislation to become effective, and the Biden administration may reverse or otherwise change these measures, both the Biden administration and Congress have indicated that they will continue to seek new legislative measures to control drug costs.

At the state level, legislatures have increasingly passed legislation and implemented regulations designed to control pharmaceutical product pricing, including price or patient reimbursement constraints, discounts, restrictions on certain product access and marketing cost disclosure and transparency measures, and, in some cases, designed to encourage importation from other countries and bulk purchasing.

Employees and human capital resources

As of December 31, 2023, we had 58 full-time employees. Of our 58 full-time employees, 44 are engaged in research and development activities. None of our employees are represented by labor unions or covered by collective bargaining agreements. We consider our relationship with our employees to be good.

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Our human capital resources objectives include, as applicable, identifying, recruiting, retaining, incentivizing and integrating our employees. We believe our success depends on our ability to attract, retain, develop and motivate diverse highly skilled personnel. In particular, we depend upon the personal efforts and abilities of the principal members of our senior management to partner effectively as a team, and to provide strategic direction, develop our business, manage our operations and maintain a cohesive and stable work environment. We also rely on qualified managers and skilled employees, such as scientists, medical professionals, engineers and laboratory technicians, with technical expertise in operations, scientific knowledge, engineering skills and quality management experience in order to operate our business successfully.

Our compensation program is designed to retain, motivate and, as needed, attract highly qualified employees. Accordingly, we use a mix of competitive base salary, performance-based equity compensation awards and other employee benefits.

Legal Proceedings

From time to time, we may become involved in legal proceedings arising in the ordinary course of our business. On October 12, 2023, Rocket filed a lawsuit in the U.S. District Court for the Southern District of New York against Lexeo, Kenneth Law and Sonia Gutierrez claiming, among other things, misappropriation of confidential information and trade secrets. The individual defendants are a current employee and a former employee of Lexeo's analytical development team, both of whom were employed at Rocket before joining Lexeo in 2021. The complaint alleges the individual defendants downloaded confidential Rocket company documents and other proprietary materials prior to leaving Rocket in 2021 and that Lexeo used this information to advance its programs. The complaint seeks unspecified damages and asks the Court to enjoin Lexeo from competing and working in the market for gene therapy treatments targeting cardiac diseases. We retained legal counsel to assist with our ongoing review of the allegations in Rocket's complaint and are confident in our defenses to the allegations. On December 7, 2023, we filed a motion to dismiss the complaint, which is now fully briefed and pending before the court. While it is not possible to predict the outcome with certainty and an estimate of the possible loss cannot be made, we currently do not expect the final outcome will have a material adverse effect on our timelines for development of our product candidates. Regardless of the final outcome, litigation can have an adverse impact on us due to defense and settlement costs, diversion of management resources, harm to our reputation and other factors.

CERTAIN RELATIONSHIPS AND RELATED PERSON TRANSACTIONS

The following is a description of each transaction since January 1, 2021, and each currently proposed transaction, in which:

- Lexeo has been or is to be a participant;
- the amount involved exceeded or exceeds \$120,000 the lesser of (a) \$120,000 and (b) 1% of the average of our total assets at year-end for the last two completed fiscal years; and
- any of Lexeo's directors, executive officers, or beneficial holders of more than 5% of any class of Legacy Lexeo voting securities, or any immediate family member of, or person sharing the household with, any of these individuals or entities, had or will have a direct or indirect material interest.

Agreements with Eric Adler, M.D.

Consulting agreement

In July 2021, in connection with the execution of the Stock Purchase Agreement with Stelios Therapeutics, Inc., or Stelios, we entered into a consulting agreement with Eric Adler, M.D., effective July 16, 2021, or the Adler Consulting Agreement, pursuant to which Dr. Adler agreed, among other things, to serve as a member of our advisory board and to advise us on *TNNI3*-associated hypertrophic cardiomyopathy, ARVC and other, to-be-determined cardiomyopathies. Dr. Adler agreed to provide consulting services of no more than six hours per week. In connection with the Adler Consulting Agreement, Dr. Adler received a cash payment of \$50,000, and we agreed to grant, subject to the approval of our board of directors, an option to purchase an aggregate of 505,366 shares to Dr. Adler. Dr. Adler provided consulting services until his appointment as our Chief Scientific Officer in August 2022.

Consultant stock option award agreements

In November 2021, we granted Dr. Adler options to purchase 39,284 shares of Common Stock at an exercise price of \$4.87 per share, and entered into the Stock Option Award Agreement, dated November 4, 2021, or the Adler Consulting Stock Option Agreement. Commencing July 16, 2021, the shares subject to the Adler Consulting Stock Option Agreement vest in 48 equal monthly installments on the first day of each month, subject to Dr. Adler's continuing to provide services to us.

Additionally, in November 2021, we granted Dr. Adler options to purchase 26,707 shares of Common Stock at an exercise price of \$4.87 per share, and entered into the Stock Option Award Agreement, dated November 9, 2021, or the Adler Performance Stock Option Agreement. All of the shares subject to the Adler Performance Stock Option Agreement vested upon our completion of the first milestone of an HCM- or ARVC-sponsored research program in June 2022.

All of the shares subject to the above options were exercisable immediately upon grant, and any then-unvested shares subject to the above options will become fully vested immediately prior to a change of control of the Company. In December 2022, our board of directors approved an amendment to Dr. Adler's Stock Option Award Agreements to extend the length of the post-termination exercise period from three months to 10 years.

Stelios Therapeutics Inc. acquisition

On July 16, 2021, we purchased all of the issued and outstanding capital stock of Stelios for initial cash consideration of \$7.0 million, with payments of up to an additional \$20.5 million due to the selling shareholders upon the achievement of the following milestones when and if we achieve them: clinical candidate selection for either the ARVC or the *TNNI3*-associated programs; first patient dosed in a Phase 1 clinical trial for any product in our CX43 program; first patient dosed in a Phase 1 clinical trial for any product in our PKP2 program; or first

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patient dosed in a Phase 1 clinical trial for any candidate in our *TNNI3* program. In the third quarter of 2022, a development milestone associated with a clinical candidate selection was achieved resulting in an aggregate payment of \$2.0 million to the selling shareholders of Stelios. Dr. Eric Adler, our Chief Medical Officer and Head of Research and an executive officer, was a co-founder of Stelios and a selling shareholder. Of the initial \$7.0 million cash consideration, Dr. Adler received approximately \$1.2 million, and of the \$2.0 million milestone payment, Dr. Adler received approximately \$0.4 million. On December 15, 2022, Stelios was merged into Lexeo Therapeutics, Inc. and ceased to exist.

Agreements with Ronald G. Crystal, M.D.

Consulting agreement

In October 2020, we entered into a consulting agreement, or the Crystal Consulting Agreement, with Ronald G. Crystal, M.D., our founder and Chief Scientific Advisor and a holder of more than 5% of our voting securities. Under the Crystal Consulting Agreement, in consideration for an annual consulting fee of \$250,000, Dr. Crystal provides us with consulting services in relation to his extensive expertise in gene therapy technologies, including (a) consulting and advisory services with respect to matters related to gene therapies for diseases; (b) participation in corporate and research and development strategy sessions; and (c) advising on recruiting and interviewing. The Crystal Consulting Agreement shall continue for five years and, unless sooner terminated in accordance with its terms, is renewable annually for one-year periods after the initial term of five years. The Crystal Consulting Agreement is terminable by either party with proper notice or by us for cause.

Stock option award agreement

In March 2021, we granted Dr. Crystal an option to purchase 169,131 shares of our Common Stock at an exercise price per share of \$2.33 and entered into our standard Stock Option Award Agreement with Dr. Crystal, or the Crystal Option Agreement. Of the shares subject to the option, 25% vested on the one-year anniversary of June 30, 2021 and the remainder of the shares subject to the option vest monthly on the thirtieth day of the month, or if there is no such date, the last day of the month, subject in each case to Dr. Crystal continuing to provide services to us. All of the shares subject to the option were exercisable immediately upon grant, and any then-unvested shares subject to the option will become fully vested as of immediately prior to a change of control of Lexeo.

Other Transactions

Investors' Rights Agreement

We are party to an amended and restated investors' rights agreement with certain holders of our capital stock, including entities affiliated with D1Capital Partners L.P., Eventide Healthcare & Life Sciences Fund, Longitude Capital Partners IV, LLC (where Reinaldo Diaz, a member of our board of directors, previously served as a Venture Partner), Lundbeckfond Invest A/S, and Omega Fund VI, L.P. (where Bernard Davitian, a member of our board of directors, serves as a Partner). Under our investors' rights agreement, certain holders of our capital stock have the right to demand that we file a registration statement or request that their shares of our capital stock be covered by a registration statement that we are otherwise filing.

Series B convertible preferred stock financing

In August 2021, we entered into a preferred stock purchase agreement with certain investors, including beneficial owners of greater than 5% of our voting securities, affiliates of members of our board of directors and certain of our executive officers, pursuant to which we issued and sold to such investors an aggregate of 58,157,823 shares of our Series B convertible preferred stock at a purchase price of \$1.72049 per share for aggregate gross proceeds of approximately \$100 million.

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The table below sets forth the aggregate number of shares of Series B convertible preferred stock issued to our related parties in this financing:

Investor	Shares Purchased (#)	Aggregate Purchase Price (\$)
D1 Master Holdco I LLC ⁽¹⁾	20,343,041	34,999,999
Entities affiliated with Janus Henderson Investors ⁽²⁾	2,324,918	3,999,998
Longitude Venture Partners IV, L.P. ⁽³⁾	2,906,148	4,999,999
Lundbeckfond Invest A/S ⁽⁴⁾	2,615,533	4,499,998
Mutual Fund Series Trust, On Behalf Of Eventide Healthcare & Life Sciences Fund ⁽⁵⁾	8,718,446	14,999,999
Omega Fund VI, L.P. ⁽⁶⁾	2,615,533	4,499,998
Paul McCormac, Ph.D. ⁽⁷⁾	29,061	49,999

- (1) D1 Master Holdco I LLC, or Holdco I, holds more than 5% of our voting securities. Holdco I is a wholly owned subsidiary of D1 Capital Partners Master LP. D1 Capital Partners L.P. is a registered investment adviser and serves as the investment manager of private investment vehicles and accounts, including D1 Capital Partners Master LP, and may be deemed to beneficially own the shares held by Holdco I. Daniel Sundheim indirectly controls D1 Capital Partners L.P., and may be deemed to beneficially own the shares held by Holdco I. Paula HJ Cholmondeley was designated to serve as a member of our board of directors by Holdco I.
- (2) Consists of (i) 883,469 shares of Series B preferred stock purchased by Janus Henderson Global Life Sciences Fund and (ii) 1,441,449 shares of Series B preferred stock purchased by Janus Henderson Capital Funds PLC on behalf of its Series Janus Henderson Global Life Sciences Fund. Entities affiliated with Janus Henderson Investors hold more than 5% of our voting securities.
- (3) Longitude Venture Partners IV, L.P., or LVP IV, holds more than 5% of our voting securities. Longitude Capital Partners IV, LLC or LCP IV is the general partner of LVP IV and may be deemed to have voting and investment power over the securities held by LVP IV. Patrick G. Enright and Juliet Tammenoms Baker are managing members of LCP IV and may be deemed to share voting and investment power over the securities held by LVP IV. LCP IV and each of these individuals disclaim beneficial ownership of such securities except to the extent of their respective pecuniary interests therein. Reinaldo Diaz was designated to serve as a member of our board of directors by LVP IV.
- (4) Lundbeckfond Invest A/S holds more than 5% of our voting securities. Mette Kirstine Agger is the former Managing Partner of Lundbeckfonden BioCapital, a department within Lundbeckfond Invest A/S, and was designated to serve as a member of our board of directors by Lundbeckfond Invest A/S.
- (5) Entities affiliated with Mutual Fund Series Trust, on behalf of Eventide Healthcare & Life Sciences Fund, hold more than 5% of our voting securities.
- (6) Omega Fund VI, L.P., or Omega Fund, holds more than 5% of our voting securities. Omega Fund VI GP, L.P., or Omega GP, is the sole general partner of Omega Fund, and Omega Fund VI GP Manager, Ltd. is the sole general partner of Omega GP. Bernard Davitian was designated to serve as a member of our board of directors by Omega Fund.
- (7) Paul McCormac previously served as an executive officer.

Participation in our Initial Public Offering

In connection with our IPO, certain of our related parties purchased shares of our Common Stock from the underwriters at the IPO price of \$11.00 per share, on the same terms as other investors in our IPO. The following table summarizes purchases of shares of our Common Stock in our IPO by our related parties:

Investor	Shares Purchased (#)	Aggregate Purchase Price (\$)
Entities affiliated with Mutual Fund Series Trust, on behalf of Eventide Healthcare & Life Sciences Fund ⁽¹⁾	2,045,454	22,499,994
Entities affiliated with Janus Henderson Investors ⁽²⁾	1,190,909	13,099,999
Longitude Venture Partners IV, L.P. ⁽³⁾	454,545	4,999,995
Omega Fund VI, L.P. ⁽⁴⁾	454,545	4,999,995
D1 Master Holdco I LLC ⁽⁵⁾	272,727	2,999,997
Lundbeckfond Invest A/S ⁽⁶⁾	227,272	2,499,992

- (1) Entities affiliated with Mutual Fund Series Trust, on behalf of Eventide Healthcare & Life Sciences Fund hold more than 5% of our voting securities.
- (2) Entities affiliated with Janus Henderson Investors hold more than 5% of our voting securities.

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- (3) LVP IV holds more than 5% of our voting securities. LCP IV is the general partner of LVP IV and may be deemed to have voting and investment power over the securities held by LVP IV. Patrick G. Enright and Juliet Tammenoms Baker are managing members of LCP IV and may be deemed to share voting and investment power over the securities held by LVP IV. LCP IV and each of these individuals disclaim beneficial ownership of such securities except to the extent of their respective pecuniary interests therein. Reinaldo Diaz was designated to serve as a member of our board of directors by LVP IV.
- (4) Omega Fund VI, L.P., or Omega Fund, holds more than 5% of our voting securities. Omega Fund VI GP, L.P., or Omega GP, is the sole general partner of Omega Fund, and Omega Fund VI GP Manager, Ltd. is the sole general partner of Omega GP. Bernard Davitian was designated to serve as a member of our board of directors by Omega Fund.
- (5) Holdco I, holds more than 5% of our voting securities. Holdco I is a wholly owned subsidiary of D1 Capital Partners Master LP. D1 Capital Partners L.P. is a registered investment adviser and serves as the investment manager of private investment vehicles and accounts, including D1 Capital Partners Master LP, and may be deemed to beneficially own the shares held by Holdco I. Daniel Sundheim indirectly controls D1 Capital Partners L.P., and may be deemed to beneficially own the shares held by Holdco I. Paula HJ Cholmondeley was designated to serve as a member of our board of directors by Holdco I.
- (6) Lundbeckfond Invest A/S holds more than 5% of our voting securities. Mette Kirstine Agger is the former Managing Partner of Lundbeckfonden BioCapital, a department within Lundbeckfond Invest A/S, and was designated to serve as a member of our board of directors by Lundbeckfond Invest A/S.

Private Placement Common Stock Purchase Agreement and Registration Rights Agreement

On March 11, 2024, we entered into a Common Stock Purchase Agreement (the “Purchase Agreement”) for a private placement (the “Private Placement”) with certain qualified institutional buyers and institutional accredited investors (each, a “Purchaser” and collectively, the “Purchasers”), which Purchasers included certain of our related parties. Pursuant to the Purchase Agreement, we agreed to sell to the Purchasers 6,278,905 Shares, at a purchase price of \$15.13 per Share.

The table below sets forth the aggregate number of shares of Common Stock issued to our related parties in the Private Placement:

Investor	Shares Purchased (#)	Aggregate Purchase Price (\$)
Citadel CEMF Investments Ltd. ⁽¹⁾	462,656	6,999,985
Mutual Fund Series Trust, on behalf of Eventide Healthcare & Life Sciences Fund ⁽²⁾	330,469	4,999,996
Longitude Venture Partners IV, L.P. ⁽³⁾	198,281	2,999,992
Omega Fund VI, L.P. ⁽⁴⁾	198,281	2,999,992

(1) Based on a Schedule 13G/A filed by Citadel Advisors LLC with the SEC on February 14, 2024, entities affiliated with Citadel CEMF Investments Ltd. held more than 5% of our voting securities.

(2) Based on a Schedule 13G filed by Eventide Asset Management, LLC with the SEC on December 11, 2023, entities affiliated with Mutual Fund Series Trust, on behalf of Eventide Healthcare & Life Sciences Fund held more than 5% of our voting securities.

(3) LVP IV holds more than 5% of our voting securities. LCP IV is the general partner of LVP IV and may be deemed to have voting and investment power over the securities held by LVP IV. Patrick G. Enright and Juliet Tammenoms Baker are managing members of LCP IV and may be deemed to share voting and investment power over the securities held by LVP IV. LCP IV and each of these individuals disclaim beneficial ownership of such securities except to the extent of their respective pecuniary interests therein.

(4) Omega Fund VI, L.P., or Omega Fund, holds more than 5% of our voting securities. Omega Fund VI GP, L.P., or Omega GP, is the sole general partner of Omega Fund, and Omega Fund VI GP Manager, Ltd. is the sole general partner of Omega GP. Bernard Davitian was designated to serve as a member of our board of directors by Omega Fund.

The Private Placement closed on March 13, 2024. The aggregate gross proceeds for the Private Placement were approximately \$95 million before deducting commissions and offering expenses payable by us. The purpose of the Private Placement was to fund working capital and other general corporate purposes.

We entered into the Registration Rights Agreement with the Purchasers, providing for the registration of the offer and resale by the Purchasers of the securities sold under the Purchase Agreement that are not then registered on an effective registration statement, pursuant to a registration statement filed with the SEC.

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We have granted the Purchasers customary indemnification rights in connection with the Registration Rights Agreement. The Purchasers have also granted us customary indemnification rights in connection with the Registration Rights Agreement.

Indemnification of Directors and Officers

Our amended and restated certificate of incorporation contains provisions that limit the liability of our current and former directors and officers for monetary damages to the fullest extent permitted by Delaware law. Delaware law provides that directors and officers of a corporation will not be personally liable for monetary damages for any breach of fiduciary duties as directors or officers (as applicable), except liability for:

- any breach of the director's or officer's duty of loyalty to the corporation or its stockholders;
- any act or omission by a director or officer not in good faith or that involves intentional misconduct or a knowing violation of law;
- a director's authorization of unlawful payments of dividends or unlawful stock repurchases or redemptions as provided in Section 174 of the Delaware General Corporation Law;
- any transaction from which the director or officer derived an improper personal benefit; or
- an action by or in the right of the corporation against an officer.

These limitations of liability do not apply to liabilities arising under federal securities laws and does not affect the availability of equitable remedies such as injunctive relief or rescission.

Our amended and restated certificate of incorporation and our amended and restated bylaws provide that we are required to indemnify our directors to the fullest extent permitted by Delaware law. Our amended and restated bylaws also provide that, upon satisfaction of certain conditions, we are required to advance expenses incurred by a director in advance of the final disposition of any action or proceeding, and permit us to secure insurance on behalf of any officer, director, employee or other agent for any liability arising out of his or her actions in that capacity regardless of whether we would otherwise be permitted to indemnify him or her under the provisions of Delaware law. Our amended and restated bylaws also provide our board of directors with discretion to indemnify our officers and employees when determined appropriate by the board.

In addition to the indemnification required in our amended and restated certificate of incorporation and amended and restated bylaws, we have entered into indemnification agreements with each of our directors and with each of our executive officers. With certain exceptions, these agreements provide for indemnification for related expenses including, among other things, attorneys' fees, judgments, fines and settlement amounts incurred by any of these individuals in any action or proceeding. We believe that these bylaw provisions and indemnification agreements are necessary to attract and retain qualified persons as directors and executive officers. We also maintain customary directors' and officers' liability insurance.

The limitation of liability and indemnification provisions in our amended and restated certificate of incorporation and amended and restated bylaws may discourage stockholders from bringing a lawsuit against our directors for breach of their fiduciary duty. They may also reduce the likelihood of derivative litigation against our directors and officers, even though an action, if successful, might benefit us and other stockholders. Further, a stockholder's investment may be adversely affected to the extent that we pay the costs of settlement and damage awards against directors and officers as required by these indemnification provisions. At present, there is no pending litigation or proceeding involving any of our directors, officers or employees for which indemnification is sought and we are not aware of any threatened litigation that may result in claims for indemnification.

Insofar as indemnification for liabilities arising under the Securities Act may be permitted for our directors, executive officers or persons controlling us, we have been informed that, in the opinion of the SEC, such indemnification is against public policy as expressed in the Securities Act and is therefore unenforceable.

Lock-Up Agreements

We, our executive officers and directors and substantially all of the holders of our Common Stock outstanding immediately prior to our IPO entered into lock-up agreements with the underwriters in our IPO or otherwise agreed, subject to limited exceptions, that we and they will not, offer, pledge, announce the intention to sell, sell, contract to sell, sell any option or contract to purchase, purchase any option or contract to sell, grant any option, right or warrant to purchase or otherwise dispose of, directly or indirectly, or enter into any swap or other agreement that transfers, in whole or in part, any of the economic consequences of ownership of our Common Stock, or any securities convertible into, or exchangeable for or that represent the right to receive shares of our Common Stock, without the prior written consent of J.P. Morgan Securities LLC and Leerink Partners LLC (the “Underwriters”) for a period of 180 days from November 2, 2023. In connection with the Private Placement, we and the Underwriters agreed to extend the lock-up agreement applicable to the Company for a period of 90 days from the Closing Date.

In addition to the restrictions contained in the lock-up agreements entered into in connection with our IPO, we entered into an agreement with the previous holders of our preferred stock (which preferred stock converted to Common Stock in connection with our IPO) that contains market stand-off provisions imposing restrictions on the ability of such security holders to sell or otherwise transfer or dispose of any registrable securities for a period of 180 days following November 2, 2023.

In connection with the Private Placement, we entered into lock-up agreements with our directors and executive officers. Subject to certain exceptions, our directors and executive officers agreed to a lock-up on their respective shares of Common Stock during the period beginning on March 11, 2024, and ending 90 days after the Closing Date.

Related Person Transactions Policy

We have adopted formal, written related person transaction policy that sets forth our procedures for the identification, review, consideration and approval or ratification of related person transactions. For purposes of our policy only, a related person transaction is a transaction, arrangement or relationship, or any series of similar transactions, arrangements or relationships, in which we and any related person are, were, or will be participants and in which the amount involved exceeds (a) \$120,000 or (b) to the extent we continue to qualify as a “smaller reporting company” under SEC rules and regulations, the lesser of (x) \$120,000 or (y) one percent of the average of our total assets at year end for the last two completed fiscal years. Transactions involving compensation for services provided to us as an employee or director are not covered by this policy. A related person is any executive officer, director or beneficial owner of more than 5% of any class of our voting securities, including any of their immediate family members and any entity owned or controlled by such persons. Prior to our IPO, we did not have a written policy for the review, approval or ratification of transactions with related parties.

Under the policy, if a transaction has been identified as a related person transaction, including any transaction that was not a related person transaction when originally consummated or any transaction that was not initially identified as a related person transaction prior to consummation, our management must present information regarding the related person transaction to our audit committee, or, if audit committee approval would be inappropriate, to another independent body of our board of directors, for review, consideration and approval or ratification. The presentation must include a description of, among other things, the material facts, the interests, direct and indirect, of the related persons, the benefits to us of the transaction and whether the transaction is on terms that are comparable to the terms available to or from, as the case may be, an unrelated third party or to or from employees generally. Under the policy, we collect information that we deem reasonably necessary from each director, executive officer and, to the extent feasible, significant stockholder to enable us to identify any existing or potential related-person transactions and to effectuate the terms of the policy. In addition, under our Code of Conduct, our employees and directors have an affirmative responsibility to disclose any transaction or relationship that reasonably could be expected to give rise to a conflict of interest. In considering

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related person transactions, our audit committee, or other independent body of our board of directors, take into account the relevant available facts and circumstances including, among other factors, (1) the risks, costs and benefits to us, (2) the impact on a director's independence in the event that the related person is a director, immediate family member of a director or an entity with which a director is affiliated, (3) the availability of other sources for comparable services or products and (4) the terms available to or from, as the case may be, unrelated third parties or to or from employees generally.

Except for the Private Placement Common Stock Purchase Agreement and Registration Rights Agreement, all of the transactions described in the above section titled "*Certain Relationships and Related Person Transactions*" were entered into prior to the adoption of this policy. Although we did not historically have a written policy for the review and approval of transactions with related persons, our board of directors historically reviewed and approved any transaction where a director or officer had a financial interest, including the transactions described in the above section titled "*Certain Relationships and Related Person Transactions*." Prior to approving such a transaction, the material facts as to a director's or officer's relationship or interest in the agreement or transaction were disclosed to our board of directors. Our board of directors took this information into account when evaluating the transaction and in determining whether such transaction was fair to us and in the best interest of all our stockholders.

MANAGEMENT

Executive Officers and Directors

The following table sets forth the names, ages, and positions of our executive officers and directors as of the date of this prospectus:

Name	Age	Position
Executive Officers		
R. Nolan Townsend	44	Chief Executive Officer and Director
Eric Adler, M.D.	51	Chief Medical Officer and Head of Research
Sandi See Tai, M.D.	53	Chief Development Officer
Jenny R. Robertson, J.D.	49	Chief Business and Legal Officer
Non-Employee Directors		
Steven Altschuler, M.D. ⁽¹⁾⁽²⁾	70	Chairman of the Board of Directors and Director
Mette Kirstine Agger ⁽¹⁾⁽³⁾	59	Director
Paula HJ Cholmondeley ⁽¹⁾⁽²⁾	76	Director
Brenda Cooperstone, M.D. ⁽²⁾⁽³⁾	59	Director
Bernard Davitian	63	Director
Reinaldo Diaz ⁽³⁾	70	Director

(1) Member of the Audit Committee.

(2) Member of the Compensation Committee.

(3) Member of the Nominating and Governance Committee.

Executive Officers

R. Nolan Townsend has served as our Chief Executive Officer and as a member of our board of directors since January 2020. Before joining Lexeo, Mr. Townsend was at Pfizer Inc., a global pharmaceutical company, from 2008 to December 2019, where he held several roles of increasing responsibility. Most recently, he served as President, Pfizer Rare Disease for the North America region. In that role, Mr. Townsend was responsible for overseeing the division's overall strategy, cross functional organization and operating budget. Mr. Townsend also currently serves on the board of Arbor Biotechnologies, a privately held genetic medicine company and on the board of directors of the Biotechnology Innovation Organization, an advocacy association representing the biotechnology industry. Mr. Townsend received a B.A. in Economics from the University of Pennsylvania and an M.B.A. from the Harvard Business School. Our board of directors believes Mr. Townsend is qualified to serve as a director because of his role as our Chief Executive Officer and his experience as an executive in the biopharmaceutical industry.

Eric Adler, M.D. has served as our Chief Medical Officer and Head of Research since February 2024. He previously served as our Chief Scientific Officer from August 2022 to February 2024. Before joining Lexeo and since July 2011, Dr. Adler has worked at the University of California, San Diego, or UCSD, where he now serves as Professor and Section Head of Heart Failure. He is also the Director of the Strauss Center for Cardiomyopathy and Czarina M. and Humberto S. Lopez Endowed Chair in Cardiology at UCSD. His work has led to the development of a novel investigational gene therapy for Danon disease, which is currently in Phase 1 clinical development. Dr. Adler's research is focused on the study and treatment of cardiomyopathy and he has published over 100 papers in peer-reviewed journals on the topic. He is currently an associate editor of Circulation Heart Failure, and he has served on leadership, grant review, and guideline committees for the American Heart Association, the Heart Failure Society of America, the International Society of Heart and Lung Transplant, and the National Institute of Health. Dr. Adler also performs clinical research on heart failure and has been a principal investigator in numerous cardiovascular clinical trials. Dr. Adler received a B.A. in English from Northwestern University and an M.D. from the Boston University School of Medicine.

Sandi See Tai, M.D., has served as the Company's Chief Development Officer since February 2024, and prior to that role served as the Company's Senior Vice President, Clinical Development and Operations from October 2023 to February 2024. Prior to joining the Company, from August 2022 to June 2023, Dr. See Tai served as the Vice President and Development Head for Rare Diseases at Pfizer Inc., a global pharmaceutical company, where she was responsible for setting the rare disease internal portfolio and business development strategy and delivering its clinical development portfolio of investigational products and in-line assets. Dr. See Tai also served in various other leading roles at Pfizer from April 2010 to August 2022, including as the Vice President and Medicine Team Lead for Rare Neuroscience, Amyloid, Rare Cardiac, Renal and Pulmonary. Prior to Pfizer, from September 2004 to March 2010, Dr. See Tai held several roles across Global Medical Affairs in Transplantation at Wyeth Pharmaceuticals Inc., a pharmaceutical company acquired by Pfizer. Prior to joining the pharmaceutical industry, from 2001 to 2004, Dr. See Tai was Assistant Professor of Pediatrics at Drexel University College of Medicine. From 2001 to 2005, Dr. See Tai was also Attending Physician in Pediatric Nephrology at St. Christopher's Hospital for Children, Philadelphia, where she completed her Pediatric Nephrology fellowship in 2001 and General Pediatrics residency in 1998.

Jenny R. Robertson, J.D. has served as our Chief Business and Legal Officer since February 2024. She previously served as our Chief Legal and Administrative Officer from July 2023 to February 2024, as our General Counsel and Company Secretary from March 2022 through December 2022 and as Senior Vice President, General Counsel and Head of People from January 2023 through June 2023. Before joining Lexeo, from April 2010 to March 2022, Ms. Robertson served in various senior legal roles at Pfizer Inc., a global pharmaceutical company, most recently as Vice President and Chief Counsel to Pfizer's Oncology Business Unit. Prior to that role, Ms. Robertson served as Chief Counsel for Pfizer's Rare Disease Business Unit from April 2016 to September 2020. From 2000 to 2010, Ms. Robertson was in private practice with Hogan & Hartson LLP. Ms. Robertson received a B.A. in Political Science from Southern Illinois University and a J.D. from the Georgetown University Law Center.

Board of Directors

Steven Altschuler, M.D. has served as the Chairman of our board of directors since January 2021. Dr. Altschuler has served as a Managing Director, Healthcare Ventures, of Ziff Capital Partners, a private investment firm, since May 2018. He previously served as a consultant to the University of Miami Health Care System from September 2017 through December 2017, the Chief Executive Officer of the University of Miami Health Care System and Executive Vice President for Healthcare at the University of Miami from January 2016 to September 2017, and the Chief Executive Officer of The Children's Hospital of Philadelphia, or CHOP, from April 2000 until June 2015. Prior to assuming the role of Chief Executive Officer, Dr. Altschuler held several positions at CHOP and the Perelman School of Medicine at the University of Pennsylvania, including Physician-in-Chief/Chair of Pediatrics and chief of the Division of Gastroenterology, Hepatology and Nutrition. Dr. Altschuler has served as a director of WW International, Inc. since September 2012, as a director of Orchard Therapeutics plc from January 2020 until it was acquired in January 2024, and as a director of 89bio, Inc. since March 2020. He previously served as Chair of the board of directors of Spark Therapeutics, Inc. from March 2013 to December 2019, as a director of Mead Johnson Nutrition from 2009 to 2019 and as a director of Adtalem Global Education Inc. from May 2018 to May 2020. Dr. Altschuler received a B.A. in Mathematics and an M.D. from Case Western Reserve University. Our board of directors believes Dr. Altschuler is qualified to serve on our board of directors based on his experience holding senior leadership positions within biotechnology companies and his role on public and private boards of directors, as well as his experience investing in healthcare companies.

Mette Kirstine Agger has served as a member of our board of directors since November 2020. Ms. Agger has served as Chief Executive Officer and Strategic Advisor of Ersum Biotech since March 2022. From 2009 to 2022, Ms. Agger served as a Senior Advisor and Managing Partner of Lundbeckfonden Biocapital (formerly Lundbeckfonden Ventures), a life sciences venture capital fund. Prior to joining Lundbeckfonden Biocapital, Ms. Agger co-founded 7TM A/S, a biotech company engaged in therapeutic drug discovery and development, in

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2000, and served as its Chief Executive Officer from founding to 2009. Ms. Agger served on the board of Trevi Therapeutics, Inc., a public life sciences company, from July 2017 to June 2019 and Veloxis Pharmaceuticals A/S, a public pharmaceutical company from April 2010 to December 2019, and has served on the board of directors of Imara Inc., a public biopharmaceutical company from January 2016 to June 2020 and scPharmaceuticals Inc., a public pharmaceutical company, since March 2014. Ms. Agger received her M.Sc. in Biology from the University of Copenhagen and received her M.B.A. from Henley Business School at the University of Reading. Our board of directors believe Ms. Agger is qualified to serve on our board of directors based on her experience holding senior leadership positions within biotechnology companies and her role on public and private boards of directors, as well as her experience investing in healthcare companies.

Paula HJ Cholmondeley has served as a member of our board of directors since November 2021. Ms. Cholmondeley has been the Chief Executive Officer of the Sorrel Group, a management consulting firm, since January 2004 and has been a part time faculty member of the National Association of Corporate Directors, or NACD, a corporate governance association, since June 2008. Ms. Cholmondeley is a NACD Certified Director and has been elected to the NACD Directorship 100. She is a former Chief Financial Officer and Certified Public Accountant. She has also held profit and loss roles as a Divisional President. Ms. Cholmondeley has served on the boards of directors of the Bank OZK, a regional bank headquartered in Little Rock, Arkansas, since May 2016, of Terex Corporation, a manufacturing company since 2004, and of Nationwide Mutual Funds, an investment firm, from 2002 to 2022. Ms. Cholmondeley holds a B.S. in Accounting from Howard University and an M.S. from the Wharton School of the University of Pennsylvania. Our board of directors believes Ms. Cholmondeley is qualified to serve on our board of directors because of her financial leadership experience and her leadership on public boards of directors.

Brenda Cooperstone, M.D. has served as a member of our board of directors since August 2023. Dr. Cooperstone has held various leadership positions at Pfizer, Inc., a global biopharmaceutical company, including as Senior Vice President since May 2017, Chief Development Officer for Rare Disease in Global Product Development from May 2016 to December 2022, and Head of Development for Rare Disease in Global Product Development from November 2015 to May 2016. Dr. Cooperstone has served as a member of the board of directors of Senti Biosciences, Inc. since October 2019 and Gandeveva Therapeutics since January 2023. Dr. Cooperstone started her career in the pharmaceutical industry at Wyeth Pharmaceuticals Inc. in 1999 and joined Pfizer, Inc., when it acquired Wyeth Pharmaceuticals, Inc., in 2009. Dr. Cooperstone earned her M.D. from McGill University and completed her residency in pediatrics at the Montreal Children's Hospital, her clinical fellowship in pediatric nephrology at Children's Hospital of Philadelphia and a research fellowship at the University of Pennsylvania's Renal Electrolyte division. Our board of directors believes Dr. Cooperstone is qualified to serve on our board of directors because of her extensive experience in the pharmaceutical industry and experience in public company board leadership.

Bernard Davitian has served as a member of our board of directors since November 2020. Mr. Davitian has been a Partner at Omega Funds, a life sciences-focused investment fund, since January 2020. Prior to that, Mr. Davitian served as Senior Vice President and Managing Director at Sanofi Ventures, the venture capital arm of Sanofi S.A., or Sanofi, for seven years from June 2012 to October 2019. Previously, he served as the Deputy of the Global Head of Business Development at Sanofi for two years. Prior to Sanofi, Mr. Davitian was Chief Financial Officer at Fovea Pharmaceuticals SA, a French biopharmaceutical R&D company specializing in ophthalmology (which was acquired by Sanofi in 2009), Chief Executive Officer at Neurotech Pharmaceuticals, Inc., a biotechnology company developing sight saving therapies for retinal diseases, and Chief Financial Officer at Transgene SA, a biopharmaceutical company. Previously, he served in various capacities at Institut Mérieux, including that of Corporate Chief Financial Officer. Prior to that, he was a senior auditor at Arthur Andersen LLP. Mr. Davitian has extensive experience in the life sciences and biotech industry, marked by a number of successful transactions involving financings, public offerings and acquisitions. Mr. Davitian is currently a board observer at Upstream Bio, where he was previously a board member from 2021 to 2024, and he has also served on the board of EyebioTech Limited since 2023, Endeavor BioMedicines since 2020 and Vanqua since 2021. Mr. Davitian previously served on the boards of Lyosomal Therapeutics from 2014 to 2019 and Click

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Therapeutics from 2018 to 2019. A Certified Public Accountant in France, Mr. Davitian holds an M.Sc. in Management (M.B.A. equivalent) from the EM Lyon Business School (France) and an A.M.P. from the Wharton School of Business at the University of Pennsylvania. Our board of directors believes Mr. Davitian is qualified to serve as a director because of his experience and leadership in healthcare venture capital investing.

Reinaldo Diaz has served as a member of our board of directors since February 2022. Mr. Diaz previously served as a Venture Partner at Longitude Capital, a healthcare venture capital firm from 2015 to 2023, and he currently serves as the Chief Executive Officer of Opna Bio, a Longitude Capital portfolio company. Mr. Diaz has also served as a Managing Director of DA Advisors, LLC since 2005, providing strategic and financial advice primarily to life science companies. From 2008 to 2018, Mr. Diaz served as a managing director at Auvon Therapeutics, a private equity firm focusing on life science companies. From 1996 to 2005, Mr. Diaz served as a managing member and co-founder of Diaz & Altschul Capital Management, LLC, an asset management firm focusing on healthcare companies. Prior to that, Mr. Diaz served as a managing director and head of the healthcare group at Schroder Wertheim & Co., Inc., and in various roles at PaineWebber Development Corporation, including as president. Mr. Diaz currently serves on the board of directors of the public pharmaceutical company, Inozyme Pharma, Inc. Mr. Diaz received a B.A. in General Studies from Harvard University and an M.B.A. from Harvard Business School. Our board of directors believes Mr. Diaz is qualified to serve as a director because of his experience in the life sciences industry and in public company board leadership.

Family Relationships

There are no family relationships among any of the executive officers or directors of the Company.

Corporate Governance Guidelines and Code of Business Conduct and Ethics

Our board of directors has adopted corporate governance guidelines. These guidelines address, among other items, the qualifications and responsibilities of our directors and director candidates, the structure and composition of our board of directors and corporate governance policies and standards applicable to us in general. In addition, our board of directors has adopted a code of business conduct and ethics that applies to all of our employees, officers and directors, including our chief executive officer, chief financial officer and other executive and senior financial officers. The full text of our corporate governance guidelines and code of business conduct and ethics are available on our website at ir.lexeotx.com. We will post amendments to our code of business conduct and ethics or any waivers of our code of business conduct and ethics for directors and executive officers on the same website.

Board of Directors

Our business and affairs are managed under the direction of our board of directors. Our board consists of seven (7) directors. The number of directors is fixed by the board of directors, subject to the terms of our amended and restated certificate of incorporation and amended and restated bylaws. Each of our directors will continue to serve as a director until the election and qualification of his or her successor, or until his or her earlier death, resignation or removal.

Classified Board of Directors

We adopted the amended and restated Certificate of Incorporation on November 7, 2023. Our certificate of incorporation provides that the board of directors is divided into three classes with staggered three-year terms. Only one Class of directors is elected at each annual meeting of our stockholders, with the other classes continuing for the remainder of their respective three-year terms. Our directors are divided among the three classes as follows:

- the Class I directors are Bernard Davitian and Mette Kirstine Agger, and their terms will expire at the annual meeting of stockholders to be held in the year that Class I director term will expire;

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- the Class II directors are Steven Altschuler, M.D., and Reinaldo Diaz, and their terms will expire at the annual meeting of stockholders to be held in the year that Class II director term will expire; and
- the Class III directors are R. Nolan Townsend, Brenda Cooperstone, M.D., and Paula HJ Cholmondeley, and their terms will expire at the annual meeting of stockholders to be held in the year that Class III director term will expire.

The Certificate of Incorporation provides that any increase or decrease in the number of directors must be distributed among the three classes so that, as nearly as possible, each Class will consist of one-third of the directors. The classification of the board of directors with staggered three-year terms may have the effect of delaying or preventing changes in control of the Company. See *"Description of Capital Stock — Anti-Takeover Provisions"*.

Director Independence

As a result of our Common Stock being listed on Nasdaq it is required to comply with the applicable rules of Nasdaq in determining whether a director is independent. Our board of directors undertook a review of its composition, the composition of its committees and the independence of our directors and considered whether any director has a material relationship with us that could compromise his or her ability to exercise independent judgment in carrying out his or her responsibilities. Based upon information requested from and provided by each director concerning his or her background, employment and affiliations, including family relationships, our board of directors has determined that Bernard Davitian, Mette Kirstine Agger, Steven Altschuler, M.D., Reinaldo Diaz, Brenda Cooperstone, M.D., and Paula HJ Cholmondeley, representing six of our seven directors, do not have a relationship that would interfere with the exercise of independent judgment in carrying out the responsibilities of a director and that each of these directors is "independent" as that term is defined under the rules of Nasdaq. R. Nolan Townsend is not an independent director because he is our Chief Executive Officer.

In making these determinations, our board of directors considered the current and prior relationships that each non-employee director has with our company and all other facts and circumstances our board of directors deemed relevant in determining their independence, including the beneficial ownership of our capital stock by each non-employee director, and the transactions involving them. There are no family relationships among any of our directors or executive officers.

Audit Committee

The audit committee consists of Ms. Cholmondeley, Ms. Agger and Dr. Altschuler. Ms. Cholmondeley is the chair of the audit committee. Our board of directors has determined that all members are independent under the listing standards of Nasdaq and Rule 10A-3(b)(1) of the Exchange Act and that Ms. Cholmondeley is an audit committee financial expert, as that term is defined under the SEC rules implementing Section 407 of the Sarbanes-Oxley Act of 2002, as amended, and possesses financial sophistication, as defined under the rules of Nasdaq. Our board of directors has also determined that each member of our audit committee can read and understand fundamental financial statements, in accordance with applicable requirements. Our audit committee oversees our corporate accounting and financial reporting process and assists our board of directors in monitoring our financial systems. Among other matters, our audit committee also:

- manages the selection, engagement, qualifications, independence and performance of a qualified firm to serve as the independent registered public accounting firm to audit our financial statements;
- discusses the scope and results of the audit with the independent registered public accounting firm, and reviews, with management and the independent accountants, our interim and year-end operating results;
- develops procedures for employees to submit concerns anonymously about questionable accounting or audit matters;

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- reviews related person transactions;
- obtains and reviews a report by the independent registered public accounting firm at least annually that describes our internal quality control procedures, any material issues with such procedures and any steps taken to deal with such issues when required by applicable law;
- approves or, as permitted, pre-approves, audit and permissible non-audit services to be performed by the independent registered public accounting firm;
- reviews and evaluates on an annual basis the performance of the audit committee and the audit committee charter; and
- any other responsibility delegated to it by the board of directors from time to time, such as oversight of our cybersecurity risk management process.

The audit committee operates under a written charter that satisfies the applicable rules of the SEC and Nasdaq listing rules.

Compensation Committee

The compensation committee consists of Dr. Cooperstone, Dr. Altschuler and Ms. Cholmondeley, each of whom meets the requirements for independence under current Nasdaq listing standards and SEC rules and regulations. Dr. Cooperstone is the chair of the compensation committee. Each member of the compensation committee is also a non-employee director, as defined pursuant to Rule 16b-3 promulgated under the Exchange Act. The compensation committee oversees our compensation policies, plans and benefits programs. Among other matters, our compensation committee also:

- reviews, modifies and approves (or if it deems appropriate, makes recommendations to the full board of directors regarding) our overall compensation strategy and policies;
- makes recommendations to the full board of directors regarding the compensation and other terms of employment of our executive officers;
- reviews and makes recommendations to the full board of directors regarding performance goals and objectives relevant to the compensation of our executive officers and assesses their performance against these goals and objectives; and
- reviews and approves (or if it deems appropriate, makes recommendations to the full board of directors regarding) the equity incentive plans, compensation plans and similar programs advisable for us, as well as modifies, amends or terminates existing plans and programs.

The compensation committee has the right, in its sole discretion, to retain or obtain the advice of compensation consultants, independent legal counsel and other advisers to assist the compensation committee in fulfilling its responsibilities under the compensation committee's written charter, only after taking into consideration the factors listed in Nasdaq Rule 5605(d)(3).

The compensation committee also has the right to take recommendations of our Chief Executive Officer into account in determining the compensation of executive officers other than our Chief Executive Officer.

The compensation committee operates under a written charter that satisfies the applicable rules of the SEC and Nasdaq listing rules.

Nominating and Corporate Governance Committee

The nominating and corporate governance committee consists of Ms. Agger, Mr. Diaz and Dr. Cooperstone, each of whom meets the requirements for independence under current Nasdaq listing standards and SEC rules

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and regulations. Ms. Agger is the chairperson of the nominating and corporate governance committee. The nominating and corporate governance committee oversees and assists our board of directors in reviewing and recommending nominees for election as directors. Among other matters, our nominating and corporate governance committee also:

- identifies, reviews and evaluates candidates to serve on our board of directors;
- determines the minimum qualifications for service on our board of directors;
- evaluates director performance on the board and applicable committees of the board and determines whether continued service on our board is appropriate;
- evaluates, nominates and recommends individuals for membership on our board of directors;
- evaluates, nominations by stockholders of candidates for election to our board of directors; and
- considers and assesses the independence of members of our board of directors.

Our nominating and corporate governance committee operates under a written charter that satisfies applicable rules of the SEC and Nasdaq listing rules.

EXECUTIVE COMPENSATION

This section provides an overview of Lexeo’s executive compensation programs, including a narrative description of the material factors necessary to understand the information disclosed in the summary compensation table below.

For the fiscal year ended December 31, 2023, Lexeo’s named executive officers were:

- R. Nolan Townsend, our Chief Executive Officer and Director;
- Jenny R. Robertson, J.D., our Chief Business and Legal Officer;
- Eric Adler, M.D., our Chief Medical Officer and Head of Research; and
- Micah Zajic, our former Chief Financial Officer and Former Chief Business Officer.

This discussion may contain forward-looking statements that are based on our current plans, considerations, expectations and determinations regarding future compensation programs. Actual compensation programs that we adopt could vary significantly from Lexeo’s historical practices and currently planned programs summarized in this discussion.

Named Executive Officers Summary Compensation Table

The following table sets forth information regarding the compensation reportable for our named executive officers for fiscal years 2023 and 2022.

<u>Name and Principal Position</u>	<u>Year</u>	<u>Salary (\$)</u>	<u>Bonus (\$)</u>	<u>Stock Awards (\$)</u>	<u>Option Awards⁽¹⁾ (\$)</u>	<u>Non-Equity Incentive Plan Compensation (\$)</u>	<u>All Other Compensation⁽²⁾ (\$)</u>	<u>Total (\$)</u>
R. Nolan Townsend	2023	508,000			872,883	347,880	8,455	1,737,218
<i>Chief Executive Officer and Director</i>	2022	465,000			—	186,000	54,432	705,432
Jenny R. Robertson, J.D.	2023	402,500			678,734	204,000	11,400	1,296,634
<i>Chief Business and Legal Officer</i>	2022	257,813	99,000		690,997	14,850	2,588	1,065,248
Eric Adler, M.D.	2023	296,500	234,750 ⁽³⁾		2,419,424	80,750	11,400	3,042,824
<i>Chief Medical Officer and Head of Research</i>								
Micah Zajic	2023	423,229	166,000 ⁽⁴⁾		2,464,231 ⁽⁵⁾		11,096	3,064,556
<i>Former Chief Financial Officer</i>								

- (1) The amounts reported consist of the aggregate grant-date fair value of restricted stock units or stock options awarded to the named executive officer, calculated in accordance with Financial Accounting Standards Board (“FASB”) Accounting Standards Codification Topic 718 (“ASC Topic 718”). The assumptions used in calculating the grant-date fair value of these awards are set forth in Notes 2 and 9 to our audited consolidated financial statements included in our Annual Report on Form 10-K, as filed with the SEC on March 11, 2024.
- (2) The amounts reported for fiscal year 2023 consist of matching contributions from the Company for the contributions made to the 401(k) plan by the Named Executive Officer and a technology stipend.
- (3) Consists of a bonus for 2023 guaranteed to Mr. Adler.
- (4) Consists of a signing bonus.
- (5) Consists of (i) an option to purchase 181,250 shares of our Common Stock pursuant to our 2021 Plan (as defined below), which was granted on March 14, 2023, and canceled on July 24, 2023, and (ii) an option to purchase 45,311 shares of our Common Stock under our 2021 Plan (the “Replacement Option”), which was granted on July 24, 2023. 45,311 shares subject to the Replacement Option expired on March 31, 2024.

Outstanding Equity Awards at Fiscal 2023 Year-End

The following table sets forth information regarding outstanding equity awards held by our named executive officers as of December 31, 2023.

Name	Grant Date ⁽¹⁾	Option Awards		Option Exercise Price(\$) ⁽²⁾	Option Expiration Date
		Number of Securities Underlying Unexercised Options Exercisable(#)	Number of Securities Underlying Unexercised Options Unexercisable(#)		
R. Nolan Townsend	2/16/2021 ⁽³⁾	153,655		\$ 2.33	2/15/2031
R. Nolan Townsend	11/15/2021 ⁽⁴⁾	519,150		\$ 4.87	11/14/2031
R. Nolan Townsend	8/22/2023 ⁽⁵⁾	106,472		\$ 11.02	8/21/2033
Jenny R. Robertson, J.D.	5/6/2022 ⁽⁶⁾	68,874		\$ 15.15	5/5/2032
Jenny R. Robertson, J.D.	3/14/2023 ⁽⁷⁾	27,187		\$ 17.59	3/13/2033
Jenny R. Robertson, J.D.	8/22/2023 ⁽⁸⁾	39,455		\$ 11.02	8/21/2033
Eric Adler, M.D.	11/4/2021 ⁽⁹⁾	39,284		\$ 4.87	11/3/2031
Eric Adler, M.D.	11/4/2021 ⁽¹⁰⁾	26,707		\$ 4.87	11/3/2031
Eric Adler, M.D.	3/14/2023 ⁽¹¹⁾	181,250		\$ 17.59	3/13/2033
Eric Adler, M.D.	8/22/2023 ⁽¹²⁾	9,438		\$ 11.02	8/21/2033
Micah Zajic	7/24/2023 ⁽¹³⁾	45,311		\$ 11.02	7/23/2033

- (1) Outstanding options to purchase shares of our Common Stock granted prior to November 2, 2023 were granted pursuant to our 2021 Plan, and outstanding options to purchase shares of our Common Stock granted on or after November 2, 2023 were granted pursuant to our 2023 Plan (as defined below).
- (2) This column represents the fair market value of a share of our Common Stock on the date of grant, as determined by our board of directors.
- (3) Vesting Commencement Date: 11/21/2020. 25% of the shares underlying the option vested on November 21, 2021, and 1/48th of the shares underlying the option vested or shall vest in monthly installments thereafter, subject to the Reporting Person's continuous service.
- (4) Vesting Commencement Date: 8/11/2021. 25% of the shares underlying the option vested on August 11, 2022, and 1/48th of the shares underlying the option vested or shall vest in monthly installments thereafter, subject to the Reporting Person's continuous service.
- (5) Vesting Commencement Date: 8/22/2023. 25% of the shares underlying the option shall vest on August 22, 2024, and 1/48th of the shares underlying the option shall vest in monthly installments thereafter, subject to the Reporting Person's continuous service.
- (6) Vesting Commencement Date: 3/21/2022. 25% of the shares underlying the option vested on March 21, 2023, and 1/48th of the shares underlying the option vested or shall vest in monthly installments thereafter, subject to the Reporting Person's continuous service.
- (7) Vesting Commencement Date: 1/1/2023. 25% of the shares underlying the option shall vest on January 1, 2024, and 1/48th of the shares underlying the option shall vest in monthly installments thereafter, subject to the Reporting Person's continuous service.
- (8) Vesting Commencement Date: 8/22/2023. 25% of the shares underlying the option shall vest on August 22, 2024, and 1/48th of the shares underlying the option shall vest in monthly installments thereafter, subject to the Reporting Person's continuous service.
- (9) Vesting Commencement Date: 7/16/2021. 1/48th of the shares underlying the option vested or shall vest in monthly installments commencing on August 1, 2021, subject to the Reporting Person's continuous service.
- (10) Vesting Commencement Date: 11/4/2021. Fully vested and exercisable as of 6/3/22.
- (11) Vesting Commencement Date: 8/1/2022. 25% of the shares underlying the option vested on August 1, 2023, and 1/48th of the shares underlying the option vested or shall vest in monthly installments thereafter, subject to the Reporting Person's continuous service.
- (12) Vesting Commencement Date: 8/22/2023. 25% of the shares underlying the option shall vest on August 22, 2024, and 1/48th of the shares underlying the option shall vest in monthly installments thereafter, subject to the Reporting Person's continuous service.
- (13) Vesting Commencement Date: 1/3/2023. 9/12 (or 75%) of the shares subject to the Option vested on September 30, 2023 and the remaining shares subject to the Option vested at a rate of 1/12 (or 8.33%) per month through December 31, 2023. 45,311 of the shares subject to the Option expired on March 31, 2024.

Employment Arrangements with our Named Executive Officers

R. Nolan Townsend

We entered into an employment agreement with Mr. Townsend, our Chief Executive Officer, on September 28, 2023. The employment agreement has no specific term and provides for at-will employment. Mr. Townsend's current annual base salary, effective as of October 1, 2023, is \$575,000.00, and Mr. Townsend's annual target bonus is 55% of his annual base salary.

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Jenny R. Robertson, J.D.

We entered into an employment agreement with Ms. Robertson, our Chief Business and Legal Officer, on September 28, 2023. The employment agreement has no specific term and provides for at-will employment. Ms. Robertson's current annual base salary, effective as of October 1, 2023, is \$425,000.00, and Ms. Robertson's annual target bonus is 40% of her annual base salary.

Eric Adler, M.D.

We previously entered into an employment agreement with Dr. Adler, our Chief Medical Officer and Head of Research, on December 21, 2022, which was superseded by an employment agreement with Dr. Adler entered into on February 3, 2024. The employment agreement has no specific term and provides for at-will employment. Dr. Adler's current annual base salary, effective as of February 15, 2024, is \$475,000, and Dr. Adler's annual target bonus is 40% of his annual base salary.

Micah Zajic

We previously entered into an employment agreement with Mr. Zajic, our former Chief Financial Officer on November 22, 2022. Mr. Zajic transitioned from his role as our Chief Financial Officer effective July 1, 2023 and served as Chief Business Officer through December 31, 2023. In connection with this transition, we entered into a transition agreement with Mr. Zajic. Pursuant to that agreement, during a period from July 1, 2023 through December 31, 2023, Mr. Zajic's employment remained at-will. Mr. Zajic's base salary was \$425,000.00. Subject to the terms of the transition agreement, Mr. Zajic was also eligible to receive a stock option to purchase 45,311 shares of our Common Stock, with 75% vesting on September 30, 2023, and 1/12th vesting per month over the following 3 months, and a discretionary bonus in an amount equal to one month of his base salary. Subject to his execution of a supplemental release agreement, he was eligible for a payment in the amount of \$5,000.

Other compensation and benefits

We maintain broad-based employee benefit plans and programs for the benefit of our employees, in which our named executive officers are entitled to participate. All of our current named executive officers are eligible to participate in our employee benefit plans, including our medical, dental, vision, and accidental death and dismemberment insurance plans, in each case on the same basis as all of our other employees.

We currently maintain a 401(k) retirement savings plan for the benefit of our employees, including Mr. Townsend, Ms. Robertson and Dr. Adler, who satisfy certain eligibility requirements. The 401(k) plan is intended to qualify as a tax-qualified plan under the Internal Revenue Code. Our named executive officers are eligible to participate in the 401(k) plan on the same basis as our other employees. The Internal Revenue Code allows eligible employees to defer a portion of their compensation, within prescribed limits, on a pre-tax basis through contributions to the 401(k) plan. Currently, we provide matching contributions of 50% on the first 6% of each participating employee's deferral up to the contribution limit permitted under the Internal Revenue Code.

Potential Payments upon Termination or Change in Control

R. Nolan Townsend

Under the employment agreement we entered into with R. Nolan Townsend, which is further described in the section titled "*Employment Arrangements with our Named Executive Officers*," if Mr. Townsend resigns for good reason or his employment is terminated without cause by us or a successor, in either case within three months prior to or within twelve months following a "change in control" (as defined in his employment agreement), Mr. Townsend will be eligible to receive the following severance benefits: (1) severance pay equal to 100% of Mr. Townsend's then-current base salary for a period of eighteen months, payable in a single lump

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sum sixty days following Mr. Townsend's last day of employment; (2) a bonus calculated at 150% of the target annual discretionary cash bonus, payable in a single lump sum within thirty days following Mr. Townsend's last day of employment; and (3) reimbursement of COBRA premiums for him and his eligible dependents from his last day of employment until the earlier of: (i) eighteen months, (ii) the time Mr. Townsend accepts employment with another employer that provides comparable benefits, or (iii) the date Mr. Townsend ceases to be eligible for COBRA continuation coverage for any reason, including plan termination. In addition, 100% of the shares subject to the options granted to Mr. Townsend will vest and become exercisable.

As a condition to receiving the severance benefits above, Mr. Townsend must sign and not revoke a general release agreement in a form reasonably acceptable to us within the time period set forth in his employment agreement and continue to comply with his obligations related to confidentiality and competitive activity.

Jenny R. Robertson, J.D.

Under the employment agreement we entered into Jenny R. Robertson, J.D. which is further described in the section titled "*Employment Arrangements with our Named Executive Officers*," if she resigns for "good reason" or we terminate her employment without "cause" (each as defined in her employment agreement), then Ms. Robertson will be eligible to receive the following severance benefits (less applicable withholdings): (1) severance pay equal to 100% of Ms. Robertson's then-current base salary for a period of twelve months, payable in a single lump sum sixty days following Ms. Robertson's last day of employment; (2) a bonus calculated at 100% of her target annual discretionary cash bonus, payable in a single lump sum sixty days following Ms. Robertson's last day of employment; and (3) reimbursement of COBRA premiums for her and her eligible dependents from her last day of employment until the earlier of: (i) twelve months, (ii) the time Ms. Robertson accepts employment with another employer that provides comparable benefits, or (iii) the date Ms. Robertson ceases to be eligible for COBRA continuation coverage for any reason, including plan termination. In addition, vesting for the shares subject to the option granted to Ms. Robertson on May 6, 2022, will be accelerated and vest by an additional four months and immediately become exercisable.

Further, if Ms. Robertson resigns for good reason or her employment is terminated without cause by us or a successor, in either case within three months prior to or within twelve months following a "change in control" (as defined in her employment agreement), Ms. Robertson will be eligible to receive the following severance benefits: (1) severance pay equal to 100% of Ms. Robertson's then-current base salary for a period of twelve months, payable in a single lump sum sixty days following Ms. Robertson's last day of employment; (2) a bonus calculated at 100% of her target annual discretionary cash bonus, payable in a single lump sum within thirty days following Ms. Robertson's last day of employment; and (3) reimbursement of COBRA premiums for her and her eligible dependents from her last day of employment until the earlier of: (i) twelve months, (ii) the time Ms. Robertson accepts employment with another employer that provides comparable benefits, or (iii) the date Ms. Robertson ceases to be eligible for COBRA continuation coverage for any reason, including plan termination. In addition, 100% of the shares subject to the options granted to Ms. Robertson will vest and become exercisable.

As a condition to receiving the severance benefits above, Ms. Robertson must sign and not revoke a general release agreement in a form reasonably acceptable to us within the time period set forth in her employment agreement and continue to comply with her obligations related to confidentiality and competitive activity.

Eric Adler, M.D.

Under the employment agreement we entered into Eric Adler, M.D., which is further described in the section titled "*Employment Arrangements with our Named Executive Officers*," if he resigns for "good reason" or we terminate his employment without "cause" (each as defined in his employment agreement), then Dr. Adler will be eligible to receive the following severance benefits (less applicable withholdings): (1) severance pay equal to 100% of his then-current base salary for a period of twelve months, payable in a single lump sum sixty

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days following Dr. Adler's last day of employment; and (2) reimbursement of COBRA premiums for him and his eligible dependents from his last day of employment until the earlier of: (i) twelve months, (ii) the time Dr. Adler becomes eligible for group health insurance through a new employer, or (iii) the date Dr. Adler ceases to be eligible for COBRA continuation coverage for any reason, including plan termination.

Further, if Dr. Adler resigns for good reason or his employment is terminated without cause by us or a successor, in either case within three months prior to or within twelve months following a "change in control" (as defined in his employment agreement), Dr. Adler will instead be eligible to receive the following severance benefits: (1) severance pay equal to 100% of his then-current base salary for a period of twelve months, payable in a single lump sum sixty days following his last day of employment; (2) a bonus calculated at 100% of his target annual discretionary cash bonus, payable in a single lump sum within thirty days following his last day of employment; and (3) reimbursement of COBRA premiums for his and his eligible dependents from his last day of employment until the earlier of: (i) twelve months, (ii) the time Dr. Adler becomes eligible for group health insurance through a new employer, or (iii) the date Ms. Robertson ceases to be eligible for COBRA continuation coverage for any reason, including plan termination. In addition, 100% of the shares subject to the options granted to Dr. Adler will vest and become exercisable.

As a condition to receiving the severance benefits above, Dr. Adler must sign and not revoke a general release agreement in a form reasonably acceptable to us within the time period set forth in his employment agreement and continue to comply with his obligations related to confidentiality and competitive activity.

Micah Zajic

Under the terms of Mr. Zajic's transition agreement, which is further described in the section titled "*Employment Arrangements with our Named Executive Officers*," in connection with the termination of his employment and execution of a supplemental release agreement, Mr. Zajic received a payment in the amount of \$5,000.

Employee Benefit and Stock Plans

2023 equity incentive plan

Our board of directors adopted the 2023 Equity Incentive Plan, or 2023 Plan, in October 2023, and our stockholders approved the 2023 Plan in October 2023. The 2023 Plan became effective on November 2, 2023.

Types of Awards. Our 2023 Plan provides for the grant of incentive stock options, or ISOs, nonstatutory stock options, or NSOs, stock appreciation rights, restricted stock awards, restricted stock unit awards, performance-based awards, and other awards, or collectively, awards. ISOs may be granted only to our employees, including our officers, and the employees of our affiliates. All other awards may be granted to our employees, including our officers, our non-employee directors, consultants, and the employees and consultants of our affiliates.

Authorized Shares. The maximum number of shares of Common Stock that may be issued under our 2023 Plan is 4,737,000 shares, which is approximately the sum of (i) 1,803,980 new shares, plus (ii) the available reserve from the 2021 Equity Incentive Plan, or the 2021 Plan, plus (iii) the number of returning shares from the 2021 Plan, if any, as such shares become available from time to time. In addition, the number of shares of Common Stock reserved for issuance under our 2023 Plan will automatically increase on January 1 of each year, beginning on January 1, 2024, and continuing through and including January 1, 2033, by 5% of the total number of shares of Common Stock outstanding on December 31 of the immediately preceding calendar year, or a lesser number of shares determined by our board of directors prior to the applicable January 1. The maximum number of shares that may be issued upon the exercise of ISOs under our 2023 Plan is 14,211,000 shares.

Shares issued under our 2023 Plan will be authorized but unissued or reacquired shares of Common Stock. Shares subject to awards granted under our 2023 Plan that expire or terminate without being exercised in full, or

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that are paid out in cash rather than in shares, will not reduce the number of shares available for issuance under our 2023 Plan. Additionally, shares issued pursuant to awards under our 2023 Plan that we repurchase or that are forfeited, as well as shares used to pay the exercise price of an award or to satisfy the tax withholding obligations to an award, will become available for future grant under our 2023 Plan.

The maximum number of shares of Common Stock subject to stock awards granted under the 2023 Plan or otherwise during any calendar year beginning in 2024 to any non-employee director, taken together with any cash fees paid by us to such non-employee director during such calendar year for service on the board of directors, will not exceed \$750,000 in total value (calculating the value of any such stock awards based on the grant date fair value of such stock awards for financial reporting purposes), or, with respect to the calendar year in which a non-employee director is first appointed or elected to our board of directors, \$1,000,000.

Plan administration. Our board of directors, or a duly authorized committee of our board, administers our 2023 Plan. Our board of directors has delegated concurrent authority to administer our 2023 Plan to the compensation committee under the terms of the compensation committee's charter. We sometimes refer to the board of directors, or the applicable committee with the power to administer our equity incentive plans, as the administrator. The administrator may also delegate to one or more of our officers the authority to (1) designate employees (other than officers) to receive specified awards, and (2) determine the number of shares subject to such awards.

The administrator has the authority to determine the terms of awards, including recipients, the exercise, purchase or strike price of awards, if any, the number of shares subject to each award, the fair market value of a share of Common Stock, the vesting schedule applicable to the awards, together with any vesting acceleration, and the form of consideration, if any, payable upon exercise or settlement of the award and the terms of the award agreements for use under our 2023 Plan.

In addition, subject to the terms of the 2023 Plan, the administrator also has the power to modify outstanding awards under our 2023 Plan, including the authority to reprice any outstanding option or stock appreciation right, cancel and re-grant any outstanding option or stock appreciation right in exchange for new stock awards, cash or other consideration, or take any other action that is treated as a repricing under generally accepted accounting principles, with the consent of any materially adversely affected participant.

Stock options. ISOs and NSOs are granted pursuant to stock option agreements adopted by the administrator. The administrator determines the exercise price for a stock option, within the terms and conditions of the 2023 Plan, provided that the exercise price of a stock option generally cannot be less than 100% of the fair market value of our Common Stock on the date of grant. Options granted under the 2023 Plan vest at the rate specified in the stock option agreement as specified in the stock option agreement by the administrator.

The administrator determines the term of stock options granted under the 2023 Plan, up to a maximum of ten years. Unless the terms of an optionholder's stock option agreement provide otherwise, if an optionholder's service relationship with us, or any of our affiliates, ceases for any reason other than disability, death, or cause, the optionholder may generally exercise any vested options for a period of three months following the cessation of service. The option term may be extended in the event that either an exercise of the option or an immediate sale of shares acquired upon exercise of the option following such a termination of service is prohibited by applicable securities laws or our insider trading policy. If an optionholder's service relationship with us or any of our affiliates ceases due to disability or death, or an optionholder dies within a certain period following cessation of service, the optionholder or a beneficiary may generally exercise any vested options for a period of 12 months in the event of disability and 18 months in the event of death. In the event of a termination for cause, options generally terminate immediately upon the termination of the individual for cause. In no event may an option be exercised beyond the expiration of its term.

Acceptable consideration for the purchase of Common Stock issued upon the exercise of a stock option will be determined by the administrator and may include (1) cash, check, bank draft, or money order, (2) a broker-

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assisted cashless exercise, (3) the tender of shares of Common Stock previously owned by the optionholder, (4) a net exercise of the option if it is an NSO, and (5) other legal consideration approved by the administrator.

Options may not be transferred to third-party financial institutions for value. Unless the administrator provides otherwise, options generally are not transferable except by will, the laws of descent and distribution or pursuant to a domestic relations order. An optionholder may designate a beneficiary, however, who may exercise the option following the optionholder's death.

Tax limitations on ISOs. The aggregate fair market value, determined at the time of grant, of Common Stock with respect to ISOs that are exercisable for the first time by an option holder during any calendar year under all of our stock plans may not exceed \$100,000. Options or portions thereof that exceed such limit will be treated as NSOs. No ISOs may be granted to any person who, at the time of the grant, owns or is deemed to own stock possessing more than 10% of our total combined voting power or that of any of our parent or subsidiary corporations, unless (1) the option exercise price is at least 110% of the fair market value of the stock subject to the option on the date of grant and (2) the term of the ISO does not exceed five years from the date of grant.

Restricted stock awards. Restricted stock awards are granted pursuant to restricted stock award agreements adopted by the administrator. Restricted stock awards may be granted in consideration for cash, check, bank draft or money order, services rendered to us, or our affiliates or any other form of legal consideration. Common stock acquired under a restricted stock award may, but need not, be subject to a share repurchase option in our favor in accordance with a vesting schedule to be determined by the administrator. A restricted stock award may be transferred only upon such terms and conditions as set by the administrator. Except as otherwise provided in the applicable award agreement, restricted stock awards that have not vested may be forfeited or repurchased by us upon the participant's cessation of continuous service for any reason.

Restricted stock unit awards. Restricted stock unit awards are granted pursuant to restricted stock unit award agreements adopted by the administrator. Restricted stock unit awards may be granted in consideration for any form of legal consideration. A restricted stock unit award may be settled by cash, delivery of stock, a combination of cash and stock as deemed appropriate by the administrator, or in any other form of consideration set forth in the restricted stock unit award agreement. Additionally, dividend equivalents may be credited in respect of shares covered by a restricted stock unit award. Except as otherwise provided in the applicable award agreement, restricted stock units that have not vested will be forfeited upon the participant's cessation of continuous service for any reason.

Stock appreciation rights. Stock appreciation rights are granted pursuant to stock appreciation right grant agreements adopted by the administrator. The administrator determines the strike price for a stock appreciation right, which generally cannot be less than 100% of the fair market value of Common Stock on the date of grant. Upon the exercise of a stock appreciation right, we will pay the participant an amount equal to the product of (1) the excess of the per share fair market value of Common Stock on the date of exercise over the strike price, multiplied by (2) the number of shares of Common Stock with respect to which the stock appreciation right is exercised. A stock appreciation right granted under the 2023 Plan vests at the rate specified in the stock appreciation right agreement as determined by the administrator.

The administrator determines the term of stock appreciation rights granted under the 2023 Plan, up to a maximum of ten years. Unless the terms of a participant's stock appreciation right agreement provide otherwise, if a participant's service relationship with us or any of our affiliates ceases for any reason other than cause, disability, or death, the participant may generally exercise any vested stock appreciation right for a period of three months following the cessation of service. The stock appreciation right term may be further extended in the event that exercise of the stock appreciation right following such a termination of service is prohibited by applicable securities laws. If a participant's service relationship with us, or any of our affiliates, ceases due to disability or death, or a participant dies within a certain period following cessation of service, the participant or a beneficiary may generally exercise any vested stock appreciation right for a period of 12 months in the event of

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disability and 18 months in the event of death. In the event of a termination for cause, stock appreciation rights generally terminate immediately upon the occurrence of the event giving rise to the termination of the individual for cause. In no event may a stock appreciation right be exercised beyond the expiration of its term.

Performance awards. Our 2023 Plan permits the grant of performance-based stock and cash awards. The administrator can structure such awards so that the stock or cash will be issued or paid pursuant to such award only following the achievement of certain pre-established performance goals during a designated performance period. Performance awards that are settled in cash or other property are not required to be valued in whole or in part by reference to, or otherwise based on, the Common Stock.

The performance goals may be based on any measure of performance selected by the board of directors. The administrator may establish performance goals on a company-wide basis, with respect to one or more business units, divisions, affiliates, or business segments, and in either absolute terms or relative to the performance of one or more comparable companies or the performance of one or more relevant indices. Unless specified otherwise (i) in the award agreement at the time the award is granted or (ii) in such other document setting forth the performance goals at the time the goals are established, the administrator will appropriately make adjustments in the method of calculating the attainment of the performance goals as follows: (1) to exclude restructuring and/or other nonrecurring charges; (2) to exclude exchange rate effects; (3) to exclude the effects of changes to generally accepted accounting principles; (4) to exclude the effects of any statutory adjustments to corporate tax rates; (5) to exclude the effects of items that are “unusual” in nature or occur “infrequently” as determined under generally accepted accounting principles; (6) to exclude the dilutive effects of acquisitions or joint ventures; (7) to assume that any business divested by us achieved performance objectives at targeted levels during the balance of a performance period following such divestiture; (8) to exclude the effect of any change in the outstanding shares of Common Stock by reason of any stock dividend or split, stock repurchase, reorganization, recapitalization, merger, consolidation, spin-off, combination or exchange of shares or other similar corporate change, or any distributions to Common Stockholders other than regular cash dividends; (9) to exclude the effects of stock-based compensation and the award of bonuses under our bonus plans; (10) to exclude costs incurred in connection with potential acquisitions or divestitures that are required to be expensed under generally accepted accounting principles; and (11) to exclude the goodwill and intangible asset impairment charges that are required to be recorded under generally accepted accounting principles.

Other awards. The administrator may grant other awards based in whole or in part by reference to Common Stock. The administrator will set the number of shares under the award and all other terms and conditions of such awards.

Changes to capital structure. In the event there is a specified type of change in our capital structure, such as a stock split, reverse stock split, or recapitalization, appropriate adjustments will be made to (1) the class and maximum number of shares reserved for issuance under the 2023 Plan; (2) the class and maximum number of shares by which the share reserve may increase automatically each year; (3) the class and maximum number of shares that may be issued upon the exercise of ISOs; and (4) the class and number of shares and exercise price, strike price, or purchase price, if applicable, of all outstanding awards.

Corporate transactions. The following applies to stock awards under the 2023 Plan in the event of a corporate transaction, unless otherwise provided in a participant’s stock award agreement or other written agreement with us or one of our affiliates or unless otherwise expressly provided by the administrator at the time of grant. Under the 2023 Plan, a corporate transaction is generally the consummation of (1) a sale or other disposition of all or substantially all of our assets, (2) a sale or other disposition of at least 50% of our outstanding securities, (3) a merger, consolidation or similar transaction following which we are not the surviving corporation, or (4) a merger, consolidation, or similar transaction following which we are the surviving corporation but the shares of Common Stock outstanding immediately prior to such transaction are converted or exchanged into other property by virtue of the transaction.

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In the event of a corporate transaction, any stock awards outstanding under the 2023 Plan may be assumed, continued or substituted for by any surviving or acquiring corporation (or its parent company), and any reacquisition or repurchase rights held by us with respect to the stock award may be assigned to the successor (or its parent company). If the surviving or acquiring corporation (or its parent company) does not assume, continue, or substitute for such stock awards, then (i) with respect to any such stock awards that are held by participants whose continuous service has not terminated prior to the effective time of the corporate transaction, or current participants, the vesting (and exercisability, if applicable) of such stock awards will be accelerated in full to a date prior to the effective time of the corporate transaction (contingent upon the effectiveness of the corporate transaction), and such stock awards will terminate if not exercised (if applicable) at or prior to the effective time of the corporate transaction, and any reacquisition or repurchase rights held by us with respect to such stock awards will lapse (contingent upon the effectiveness of the corporate transaction), and (ii) any such stock awards that are held by persons other than current participants will terminate if not exercised (if applicable) prior to the effective time of the corporate transaction, except that any reacquisition or repurchase rights held by us with respect to such stock awards will not terminate and may continue to be exercised notwithstanding the corporate transaction. In addition, the plan administrator may also provide, in its sole discretion, that the holder of a stock award that will terminate upon the occurrence of a corporate transaction if not previously exercised will receive a payment, if any, equal to the excess of the value of the property the participant would have received upon exercise of the stock award over the exercise price otherwise payable in connection with the stock award.

A stock award may be subject to additional acceleration of vesting and exercisability upon or after a change in control as may be provided in an applicable award agreement or other written agreement, but in the absence of such provision, no such acceleration will occur.

Transferability. A participant may not transfer awards under our 2023 Plan other than by will, the laws of descent and distribution, or as otherwise provided under our 2023 Plan.

Plan amendment or termination. Our board of directors has the authority to amend, suspend, or terminate our 2023 Plan, provided that such action does not materially impair the existing rights of any participant without such participant's written consent. Certain material amendments also require the approval of our stockholders. No ISOs may be granted after the tenth anniversary of the date our board of directors adopted our 2023 Plan. No awards may be granted under our 2023 Plan while it is suspended or after it is terminated.

2023 employee stock purchase plan

Our board of directors adopted the 2023 ESPP in October 2023, and our stockholders adopted the 2023 ESPP in October 2023. The 2023 ESPP became effective on November 2, 2023. The purpose of the 2023 ESPP is to secure the services of new employees, to retain the services of existing employees and to provide incentives for such individuals to exert maximum efforts toward our success and that of our affiliates. The 2023 ESPP includes two components. One component is designed to allow our eligible U.S. employees to purchase Common Stock in a manner that may qualify for favorable tax treatment under Section 423 of the Internal Revenue Code of 1986, as amended, or the Code. In addition, purchase rights may be granted under a component that does not qualify for such favorable tax treatment when necessary or appropriate to permit participation by our eligible employees who are foreign nationals or employed outside of the United States while complying with applicable foreign laws.

Authorized shares. The maximum aggregate number of shares of Common Stock that may be issued under our 2023 ESPP is 238,600 shares. The number of shares of Common Stock reserved for issuance under our 2023 ESPP will automatically increase on January 1 of each calendar year, beginning on January 1, 2024 and continuing through and including January 1, 2033, by the lesser of (1) 1% of the total number of shares of capital stock outstanding on December 31 of the preceding calendar year, (2) 477,200 shares and (3) a number of shares determined by our board of directors. Shares subject to purchase rights granted under our 2023 ESPP that terminate without having been exercised in full will not reduce the number of shares available for issuance under our 2023 ESPP.

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Plan administration. Our board of directors, or a duly authorized committee thereof, administers our 2023 ESPP. Our board of directors has delegated concurrent authority to administer our 2023 ESPP to the compensation committee under the terms of the compensation committee's charter. The 2023 ESPP is implemented through a series of offerings with specific terms approved by the administrator and under which eligible employees are granted purchase rights to purchase shares of Common Stock on specified dates during such offerings. Under the 2023 ESPP, we may specify offerings with durations of not more than 27 months and may specify shorter purchase periods within each offering. Each offering will have one or more purchase dates on which shares of Common Stock will be purchased for our eligible employees participating in the offering. An offering under the 2023 ESPP may be terminated under certain circumstances.

Payroll deductions. Generally, all regular employees, including executive officers, employed by us or by any of our designated affiliates, may participate in the 2023 ESPP and may contribute, normally through payroll deductions, with a maximum dollar amount as designated by the board of directors. Unless otherwise determined by the administrator, Common Stock will be purchased for the accounts of employees participating in the 2023 ESPP at a price per share equal to the lower of (a) 85% of the fair market value of a share of Common Stock on the first date of an offering or (b) 85% of the fair market value of a share of Common Stock on the date of purchase. For the initial offering, which we expect will commence upon the execution and delivery of the underwriting agreement relating to this offering, the fair market value on the first day of the initial offering will be the price at which shares are first sold to the public.

Limitations. Our employees, including executive officers, or any of our designated affiliates may have to satisfy one or more of the following service requirements before participating in our 2023 ESPP, as determined by the administrator: (1) customary employment with us or one of our affiliates for more than 20 hours per week and more than five months per calendar year, or (2) continuous employment with us or one of our affiliates for a minimum period of time, not to exceed two years, prior to the first date of an offering. An employee may not be granted rights to purchase stock under our 2023 ESPP if such employee (1) immediately after the grant would own stock possessing 5% or more of the total combined voting power or value of Common Stock, or (2) holds rights to purchase stock under our 2023 ESPP that would accrue at a rate that exceeds \$25,000 worth of our stock for each calendar year that the rights remain outstanding.

Changes to capital structure. In the event that there occurs a change in our capital structure through such actions as a stock split, merger, consolidation, reorganization, recapitalization, reincorporation, stock dividend, dividend in property other than cash, large nonrecurring cash dividend, liquidating dividend, combination of shares, exchange of shares, change in corporate structure or similar transaction, the board of directors will make appropriate adjustments to (1) the number of shares reserved under the 2023 ESPP, (2) the maximum number of shares by which the share reserve may increase automatically each year, (3) the number of shares and purchase price of all outstanding purchase rights, and (4) the number of shares that are subject to purchase limits under ongoing offerings.

Corporate transactions. In the event of certain corporate transactions, including: (1) a sale of all or substantially all of our assets, (2) the sale or disposition of 50% of our outstanding securities, (3) the consummation of a merger or consolidation where we do not survive the transaction, and (4) the consummation of a merger or consolidation where we do survive the transaction but the shares of our Common Stock outstanding immediately before such transaction are converted or exchanged into other property by virtue of the transaction, any then-outstanding rights to purchase our stock under the 2023 ESPP may be assumed, continued or substituted for by any surviving or acquiring entity (or its parent company). If the surviving or acquiring entity (or its parent company) elects not to assume, continue, or substitute for such purchase rights, then the participants' accumulated payroll contributions will be used to purchase shares of Common Stock within 10 business days (or such other period specified by the board) prior to such corporate transaction and such purchase rights will terminate immediately.

Under the 2023 ESPP, a corporate transaction is generally the consummation of: (1) a sale of all or substantially all of our assets, (2) the sale or disposition of more than 50% of our outstanding securities, (3) a

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merger or consolidation where we do not survive the transaction, and (4) a merger or consolidation where we do survive the transaction but the shares of our Common Stock outstanding immediately before such transaction are converted or exchanged into other property by virtue of the transaction.

2023 ESPP amendment or termination. The administrator has the authority to amend or terminate our 2023 ESPP, provided that except in certain circumstances such amendment or termination may not materially impair any outstanding purchase rights without the holder's consent. We will obtain stockholder approval of any amendment to our 2023 ESPP as required by applicable law or listing requirements.

2021 equity incentive plan

Our board of directors adopted the 2021 Plan in February 2021, and our stockholders approved the 2021 Plan in September 2021.

Stock awards. The 2021 Plan provides for the grant of ISOs, NSOs, and restricted stock awards, or collectively, stock awards. ISOs may be granted only to our employees and the employees of any parent corporation or subsidiary corporation. All other awards may be granted to our service providers. We have granted ISOs and NSOs under the 2021 Plan. As of June 30, 2023, options to purchase 1,873,093 shares of our Common Stock were outstanding with a weighted average exercise price of \$7.94 per share, with 30,516 restricted shares of our Common Stock issued remaining subject to our right of repurchase, and 1,039,784 shares of our Common Stock remained available for future awards under the 2021 Plan.

Share reserve. The 2021 Plan was terminated in November 2023, and no additional awards will be granted thereunder. The 2021 Plan continues to govern outstanding awards granted thereunder.

Shares subject to stock awards granted under the 2021 Plan that expire or are forfeited or become unexercisable without having been exercised in full, or are surrendered pursuant to an exchange program shall continue to be available under the 2021 Plan for issuance pursuant to future awards. If any shares of Common Stock issued pursuant to a stock award are forfeited back to or repurchased by us for at the original purchase price, the shares that are forfeited or repurchased or reacquired will revert to and again become available for issuance under the 2021 Plan. Any shares retained in satisfaction of tax withholding obligations or as consideration for the exercise or purchase price of a stock award will again become available for issuance under the 2021 Plan.

Plan administration. Our board of directors administers and interprets the provisions of the 2021 Plan. The board of directors may delegate its authority to a committee, or committees, of the board, referred to as the "administrator." The administrator may additionally delegate limited authority to specified directors or executive officers. Under the 2021 Plan, the administrator has the authority to, among other things, approve award recipients, determine the numbers and types of stock awards to be granted, determine the applicable fair market value and the provisions of each stock award, including the period of their exercisability and the vesting schedule applicable to a stock award, construe and interpret the 2021 Plan and awards granted thereunder, and prescribe, amend, modify, and rescind or terminate rules and regulations for the administration of the 2021 Plan.

Stock options. ISOs and NSOs are granted under stock option agreements in such form and containing such provisions as approved by the administrator. The administrator determines the exercise price for stock options, within the terms and conditions of the 2021 Plan, provided that the exercise price of a stock option generally will not be less than 100% of the fair market value of our Common Stock on the date of grant (or 110% of the fair market value for 10% stockholders as required by the Code). Stock options granted under the 2021 Plan vest at the rate specified in the stock option agreements and option rules as determined by the administrator.

The administrator determines the term of stock options granted under the 2021 Plan, up to a maximum of 10 years (or five years for 10% stockholders as required by the Code). If an optionholder's service relationship

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with us or any of our affiliates ceases for any reason other than disability, death, or cause the optionholder may generally exercise any vested options for a period of up to three months following the cessation of service, or such other period of time set forth in the option agreement. If an optionholder's service relationship with us or any of our affiliates ceases due to death or disability (or the participant dies within three months after a termination other than for cause), then options vested as of the termination date may generally be exercised within 12 months following the date of termination, or such other period of time set forth in the option agreement. In no event may an option be exercised beyond the expiration of its term. If an optionholder's service relationship with us or any of our affiliates ceases due to termination for cause, the optionholder's vested options shall expire on the optionholder's termination date, or such later time as determined by the administrator.

The exercise price for shares issued under the 2021 Plan are generally payable in cash, check, promissory note, surrender of previously owned shares, consideration received through a broker-assisted (or other) cashless exercise program (whether through a broker or otherwise) implemented by us, net exercise, or other forms of consideration determined by the administrator (or any combination thereof).

Unless the administrator provides otherwise, options generally are not transferable except by will, the laws of descent and distribution, or, with respect to NSOs for participants in the United States, by gift to a family member.

Restricted stock. The administrator determines to whom an offer of restricted stock will be made, the number of shares the person may purchase, the purchase price, the restrictions to which the shares will be subject, and other terms and conditions. If a participant's service relationship with us ends for any reason, we may receive any or all of the shares of Common Stock held by the participant that have not vested as of the date the participant terminates service with us through, but not limited to, a repurchase right.

Changes to capital structure. In the event of any stock split, reverse stock split, stock dividend, combination, consolidation, recapitalization (including a recapitalization through a large nonrecurring cash dividend) or reclassification of the shares, subdivision of the shares, a rights offering, a reorganization, merger, spin-off, split-up, repurchase, or exchange of our Common Stock or other securities of ours or other significant corporate transaction, or other change affecting our Common Stock, then in order to prevent diminution or enlargement of the benefits or potential benefits intended to be made available under the 2021 Plan, the administrator will adjust the number, kind and class of securities that may be delivered under the 2021 Plan and/or the number, class, kind and price of securities covered by each outstanding award.

Change in control. In the event of Change in Control under the 2021 Plan shall be subject to the agreement evidencing the acquisition or other combination, which need not treat all outstanding awards in an identical manner. Such agreement, without the participant's consent, shall provide for one or more of the following with respect to all outstanding awards:

- the continuation of such outstanding awards;
- the assumption of such outstanding awards by the surviving corporation or its parent;
- the substitution by the surviving corporation or its parent of new options or other equity awards for such awards;
- the cancellation of such awards in exchange for a payment to the participants equal to the excess, if any, of (1) the fair market value of the shares subject to such awards as of the closing date of such Change in Control over (2) the exercise or purchase price paid or to be paid for the shares subject to the awards; provided that at the discretion of the administrator, such payment may be subject to the same conditions that apply to the consideration that will be paid to holders of shares in connection with the transaction; or
- the opportunity for participants to exercise the options prior to the occurrence of the Change in Control and the termination (for no consideration) upon the consummation of such Change in Control of any options not exercised prior thereto.

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Under the 2021 Plan, a Change in Control is generally defined as (i) the consummation of a merger or consolidation of the company with or into another entity or any other corporate reorganization, if the company's stockholders immediately prior to such merger, consolidation or reorganization cease to directly or indirectly own immediately after such merger, consolidation or reorganization at least a majority of the combined voting power of the continuing or surviving entity's securities outstanding immediately after such merger, consolidation or reorganization; (ii) the consummation of the sale, transfer or other disposition of all or substantially all of the company's assets; (iii) a change in the effective control of the company; or (iv) the consummation of any transaction as a result of which any Person (as defined below) becomes the "beneficial owner" (as defined in Rule 13d-3 under the Exchange Act), directly or indirectly, of securities of the company representing at least fifty percent (50%) of the total voting power represented by the company's then outstanding voting securities. For purposes of this Section 2(h), the term "Person" shall have the same meaning as when used in Sections 13(d) and 14(d) of the Exchange Act but shall exclude:

- a trustee or other fiduciary holding securities under an employee benefit plan of the company or an affiliate of the company;
- a corporation or other entity owned directly or indirectly by the stockholders of the company in substantially the same proportions as their ownership our Common Stock;
- the company; and
- a corporation or other entity of which at least a majority of its combined voting power is owned directly or indirectly by the company.

A transaction shall not constitute a Change in Control if its sole purpose is to change the state of the company's incorporation or to create a holding company that will be owned in substantially the same proportions by the persons who held the company's securities immediately before such transactions. In addition, if any Person (as defined above) is considered to be in effective control of the company, the acquisition of additional control of the company by the same Person will not be considered to cause a Change in Control. If required for compliance with Section 409A of the Code, in no event will a Change in Control be deemed to have occurred if such transaction is not also a "change in the ownership or effective control of" the company or "a change in the ownership of a substantial portion of the assets of" the company as determined under Treasury Regulation Section 1.409A-3(i)(5) (without regard to any alternative definition thereunder).

Plan Amendment or Termination. The board of directors may at any time terminate, amend or suspend the 2021 Plan and all outstanding options or restricted stock awards upon a dissolution or liquidation of us.

Non-Employee Director Compensation Policy

We adopted a non-employee director compensation policy (the "Director Compensation Policy") that became effective upon the closing of our IPO in November 2023, and may be amended at any time in the sole discretion of the board of directors or the compensation committee. Under our Director Compensation Policy as currently in effect, each non-employee and non-consultant director (each, an "Eligible Director") upon first becoming a director receives an initial option to purchase 36,000 shares of Common Stock. The initial option vests over a three-year period in equal annual installments after the grant date, subject to continued service through the vesting date. Additionally, each Eligible Director automatically receives an annual option to purchase 18,000 shares (the "Annual Grant"), effective on the date of each annual meeting of the stockholders. In addition, each Eligible Director who is first elected to the board of directors other than at an annual meeting of the stockholders receives an Annual Grant, pro-rated for the number of months remaining until the next annual meeting of the stockholders. The Annual Grant vests on the earlier of one year following the grant date or the next annual meeting of stockholders, subject to continued service through the vesting date. All awards under the Director Compensation Policy accelerate and vest upon a change in control. The exercise price of all options under the Director Compensation Policy is the fair market value on the date of grant.

[Table of Contents](#)**Cash Compensation**

All non-employee directors are entitled to receive the following cash compensation for their services:

Position	Annual Cash Retainer
<i>Base Director Fee</i>	\$ 40,000
<i>Additional Chairperson Fee</i>	
Chair of the Board of Directors	\$ 30,000
Chair of the Audit Committee	\$ 15,000
Chair of the Compensation Committee	\$ 12,000
Chair of the Nominating and Corporate Governance Committee	\$ 12,000
<i>Additional Committee Member Fee (excluding chairpersons)</i>	
Audit Committee	\$ 7,500
Compensation Committee	\$ 6,000
Nominating and Corporate Governance Committee	\$ 6,000

Director Compensation for Fiscal Year Ending 2023

The following table sets forth information regarding the total compensation awarded to, earned by or paid to our non-employee directors for their service on our board of directors, for the fiscal year ending December 31, 2023. Directors who are also our employees receive no additional compensation for their service as directors. For the fiscal year ending December 31, 2023, Mr. Townsend was an employee and executive officer of the Company and therefore, did not receive compensation as a director. See “*Executive Compensation*” for additional information regarding Mr. Townsend’s compensation.

Name	Fees Paid or Earned in Cash (\$)	Stock Awards (\$)	Option Awards (\$)	Non-Equity Incentive Plan Compensation (\$)	Nonqualified Deferred Compensation Earnings (\$)	All Other Compensation (\$)	Total (\$)
Bernard Davitian	\$ 6,413.04	—	—	—	—	—	\$ 6,413.04
Mette Kirstine Agger	\$ 10,501.36	—	—	—	—	—	\$ 10,501.36
Steven Altschuler, M.D.	\$139,220.56	—	\$ 60,932.60	—	—	—	\$200,153.16
Reinaldo Diaz	\$ 7,375.00	—	—	—	—	—	\$ 7,375.00
Brenda Cooperstone, M.D.	\$ 18,907.60	—	\$493,102.51	—	—	\$ 17,125.00	\$529,135.11
Paula HJ Cholmondeley	\$ 42,050.27	—	\$364,555.56	—	—	—	\$406,605.83

The following table lists all outstanding equity awards held by non-employee directors as of December 31, 2023.

Name	Number of Shares Underlying Outstanding Stock Awards	Number of Shares Underlying Outstanding Options
Steven Altschuler, M.D.	—	119,625
Brenda Cooperstone, M.D.	—	59,812
Paula HJ Cholmondeley	7,489	44,184

Indemnification

We have entered into an indemnification agreement with each of our directors and executive officers. The indemnification agreements and our amended and restated certificate of incorporation and amended and restated bylaws require us to indemnify our directors and executive officers to the fullest extent permitted by Delaware law. See “*Certain Relationships and Related Person Transactions—Indemnification of Directors and Officers.*”

PRINCIPAL AND SELLING STOCKHOLDERS

The following table sets forth:

- the beneficial ownership of our capital stock as of April 2, 2024 (unless otherwise specified), as adjusted to reflect the Common Stock that may be sold from time to time pursuant to this prospectus, for: (i) each person or group of affiliated persons known to us to be the beneficial owner of more than 5% of outstanding Common Stock; (ii) each of our named executive officers and directors; and (iii) all of our executive officers and directors as a group.
- certain information concerning the shares of Common Stock that may be offered from time to time by each Selling Stockholder under this prospectus.

This prospectus relates to the resale of up to 6,974,248 shares of our Common Stock held by the Selling Stockholders.

The Selling Stockholders may from time to time offer and sell any or all of the shares of Common Stock set forth below pursuant to this prospectus and any accompanying prospectus supplement. When we refer to the “Selling Stockholders” in this prospectus, we mean the persons listed in the table below, and the pledgees, donees, transferees, assignees, successors, designees and others who later come to hold any of the Selling Stockholders’ interest in the Common Stock other than through a public sale.

The following table sets forth, as of the date of this prospectus, the names of the Selling Stockholders, and the aggregate number of shares of Common Stock that the Selling Stockholders may offer pursuant to this prospectus. For purposes of this table, we have assumed that the Selling Stockholders will have sold all of the securities covered by this prospectus upon the completion of the offering.

We cannot advise you as to whether the Selling Stockholders will in fact sell any or all of such securities. In particular, the Selling Stockholders identified below may have sold, transferred or otherwise disposed of all or a portion of their shares after the date on which they provided us with information regarding their securities. Any changed or new information given to us by the Selling Stockholders, including regarding the identity of, and the securities held by, each Selling Stockholder, will be set forth in a prospectus supplement or amendments to the registration statement of which this prospectus is a part, if and when necessary.

Please see the section entitled “*Plan of Distribution*” for further information regarding the Selling Stockholders’ method of distributing these securities.

We have determined beneficial ownership in accordance with the rules and regulations of the SEC and the information is not necessarily indicative of beneficial ownership for any other purpose. Except as indicated by the footnotes below, we believe, based on information furnished to us, that the persons and entities named in the table below have sole voting and sole investment power with respect to all shares that they beneficially own, subject to applicable community property laws.

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Unless otherwise indicated, the address of each beneficial owner listed in the table below is c/o Lexeo Therapeutics, Inc., 345 Park Avenue South, Floor 6, New York, New York, 10010.

Name of Beneficial Owner	Shares Beneficially Owned Prior to this Offering		Shares Being Offered	Shares Beneficially Owned After this Offering	
	Number of Shares	Percentage		Number of Shares	Percentage
<i>Greater than 5% Stockholders:</i>					
Citadel CEMF Investments Ltd. and affiliates ⁽¹⁾	2,504,412	7.6	462,656	2,041,756	6.2
D1 Capital partners L.P. ⁽²⁾	2,506,607	9.4	—	2,506,607	9.4
Eventide Asset Management, LLC ⁽³⁾	3,369,147	10.2	330,469	3,038,678	9.2
Janus Henderson Group plc ⁽⁴⁾	2,330,738	8.7	—	2,330,738	8.7
Longitude Capital Partners IV, LLC ⁽⁵⁾	2,765,381	8.4	198,281	2,567,100	7.8
Lundbeckfond Invest A/S ⁽⁶⁾	1,835,959	6.9	—	1,835,959	6.9
Omega Fund VI, L.P. ⁽⁷⁾	2,355,904	7.2	198,281	2,157,623	6.5
<i>Named Executive Officers and Directors:</i>					
R. Nolan Townsend ⁽⁸⁾	906,222	2.7	—	906,222	2.7
Eric Adler, M.D. ⁽⁹⁾	256,679	0.8	—	256,679	0.8
Jenny R. Robertson, J.D. ⁽¹⁰⁾	135,516	0.4	—	135,516	0.4
Micah Zajic	—	*	—	—	*
Mette Kirstine Agger	—	*	—	—	*
Steven Altschuler, M.D. ⁽¹¹⁾	119,625	0.4	—	119,625	0.4
Paula HJ Cholmondeley ⁽¹²⁾	59,811	0.2	—	59,811	0.2
Brenda Cooperstone, M.D. ⁽¹³⁾	59,812	0.2	—	59,812	0.2
Bernard Davitian	—	*	—	—	*
Reinaldo Diaz	—	*	—	—	*
All current executive officers and directors (10 persons) ⁽¹⁴⁾	1,537,665	4.5	—	1,537,665	4.5
<i>Other Selling Stockholders:</i>					
Adage Capital Partners, L.P. ⁽¹⁵⁾	1,247,736	3.8	1,222,736	25,000	0.1
Allostery Master Fund LP ⁽¹⁶⁾	123,046	0.4	33,046	90,000	0.3
Braidwell Partners Master Fund LP ⁽¹⁷⁾	1,322,277	4.0	1,321,877	400	*
Gray's Creek Capital Partners Fund I, LP ⁽¹⁸⁾	128,784	0.4	33,046	95,738	0.3
Integrated Core Strategies (US) LLC ⁽¹⁹⁾	783,694	2.4	462,656	321,038	1.0
Invus Public Equities, L.P. ⁽²⁰⁾	801,716	2.4	198,281	603,435	1.8
Irving Investors, LLC ⁽²¹⁾	99,140	0.3	99,140	—	*
Laurion Capital Master Fund Ltd ⁽²²⁾	454,182	1.4	198,281	255,901	0.8
Novo Holdings A/S ⁽²³⁾	1,638,298	5.0	330,469	1,307,829	4.0
Octagon Investments Master Fund LP ⁽²⁴⁾	132,187	0.4	132,187	—	*
RA Capital Healthcare Fund, L.P. ⁽²⁵⁾	660,938	2.0	660,938	—	*
Entities Affiliated with Vestal Point Capital, LP ⁽²⁶⁾	175,000	0.5	165,234	9,766	*
Woodline Master Fund LP ⁽²⁷⁾	794,483	2.4	794,483	—	2.1
Xantium Partners L.P. ⁽²⁸⁾	132,187	0.4	132,187	—	*

* Represents beneficial ownership or voting power of less than one percent.

(1) Represents shares held by Citadel CEMF Investments Ltd. ("CCIL") and affiliates. "Shares Beneficially Owned Prior to the Offering" includes (i) 462,656 shares held by CCIL, a selling stockholder, and (ii) 2,041,756 shares held by affiliates of CCIL, including Citadel Multi-Strategy Equities Master Fund Ltd. ("CEMF"), which holds 2,038,606 shares. Citadel Advisors LLC ("Citadel Advisors") is the portfolio manager of CCIL and CEMF. Citadel Advisors Holdings LP ("CAH"), is the sole member of Citadel Advisors. Citadel GP LLC ("CGP"), is the general partner of CAH. Kenneth Griffin owns a controlling interest in CGP. Mr. Griffin, as the owner of a

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controlling interest in CGP, may be deemed to have shared power to vote or direct the vote of, and/or shared power to dispose or to direct the disposition over, the shares beneficially owned reported here. "Shares to be Sold in this Offering" are shares held by CCIL. This disclosure is not and shall not be construed as an admission that Mr. Griffin or any of the entities listed above is the beneficial owner of any securities of the Company other than the securities actually owned by such person (if any). The address of CCIL and CEMF is c/o Citadel Enterprise Americas LLC, Southeast Financial Center, 200 S. Biscayne Blvd., Suite 3300, Miami, Florida 33131.

- (2) According to the Schedule 13G filed on February 14, 2024. Represents shares held by D1 Capital Partners, L.P. ("D1 L.P."), which may be deemed to have shared voting and shared dispositive power with Daniel Sundheim. D1 L.P. is a registered investment adviser and serves as the investment manager of private investment vehicles and accounts, including D1 Capital Partners Master LP (the "Investment Vehicle"), and may be deemed to beneficially own the shares held by the Investment Vehicle and/or its subsidiary. Mr. Sundheim indirectly controls the Investment Manager and may be deemed to beneficially own the shares held by the Investment Vehicle and/or its subsidiary. The address for each of D1 L.P. and Mr. Sundheim is 9 West 57th Street, 36th Floor, New York, New York 10019.
- (3) Represents shares held by Mutual Fund Series Trust, on behalf of Eventide Healthcare & Life Sciences Fund. Eventide Healthcare & Life Sciences Fund is a registered investment company for which Eventide Asset Management, LLC acts as investment advisor. Eventide Asset Management, LLC has voting and investment power with respect to the shares. The address of Mutual Fund Series Trust is Mutual Fund Series Trust, O/B/O Eventide Healthcare & Life Sciences Fund, 80 Arkay Drive, Suite 110, Hauppauge, New York 11788.
- (4) According to the Schedule 13G filed on February 14, 2024. Represents shares held by Janus Henderson Group plc ("Janus Henderson") and certain of its subsidiaries. The address of Janus Henderson is 201 Bishopsgate EC2M 3AE, United Kingdom.
- (5) Represents shares held by Longitude Venture Partners IV, L.P. ("LVP IV"). Longitude Capital Partners IV, LLC (LCP IV), is the general partner of LVP IV and may be deemed to have voting and investment power over the shares held by LVP IV. Patrick Enright and Juliet Tammenoms Bakker are managing members of LCP IV and may be deemed to share voting and investment power over the shares held by LVP IV. Each of LCP IV, Ms. Tammenoms Bakker and Mr. Enright disclaims beneficial ownership of such shares except to the extent of their respective pecuniary interests therein. The address of LVP IV is 2740 Sand Hill Road, 2nd Floor, Menlo Park, California 94025.
- (6) According to the Schedule 13G filed on February 14, 2024. Represents shares held by Lundbeckfond Invest A/S ("Lundbeckfond"), for which Lene Skole, Chief Executive Officer of Lundbeckfond, may be deemed to have sole voting and disposal power. The address of Lundbeckfond is Scherfigsvej 7 DK-2100, København Ø, Denmark.
- (7) Represents shares held by Omega Fund VI, L.P. ("Omega Fund"). Omega Fund VI GP Manager, Ltd. ("Omega Ltd.") is the sole general partner of Omega Fund VI GP, L.P. ("Omega GP"), which is the sole general partner of Omega Fund. Each of Omega Ltd. and Omega GP may be deemed to own beneficially the shares held by Omega Fund. Otello Stampacchia, Claudio Nessi and Anne-Mari Paster are the directors of Omega Ltd., and, as a result, may be deemed to share voting and investment power over the shares held directly by Omega Fund. Each of Dr. Stampacchia, Dr. Nessi, Ms. Paster, Omega Ltd. and Omega GP disclaims beneficial ownership of the shares held by Omega Fund except to the extent of their pecuniary interest therein. The address for Omega Fund is 888 Boylston Street, Suite 1111, Boston, Massachusetts 02199.
- (8) Represents (i) 126,945 shares of Common Stock and (ii) 779,277 shares of Common Stock issuable to Mr. Townsend upon exercise of outstanding options that are exercisable within 60 days of April 2, 2024, including 294,317 unvested shares which may be acquired through early option exercise.
- (9) Represents 256,679 shares of Common Stock issuable to Dr. Adler upon exercise of outstanding options that are exercisable within 60 days of April 2, 2024, including 118,256 unvested shares which may be acquired through early option exercise.
- (10) Represents 135,516 shares of Common Stock issuable to Ms. Robertson upon exercise of outstanding options that are exercisable within 60 days of April 2, 2024, including 88,583 unvested shares which may be acquired through early option exercise.
- (11) Represents 119,625 shares of Common Stock issuable to Dr. Altschuler upon exercise of outstanding options that are exercisable within 60 days of April 2, 2024, including 24,708 unvested shares which may be acquired through early option exercise.
- (12) Represents (i) 15,627 shares of Common Stock and (ii) 44,184 shares of Common Stock issuable to Ms. Cholmondeley upon exercise of outstanding options that are exercisable within 60 days of April 2, 2024, including 35,900 unvested shares which may be acquired through early option exercise.
- (13) Represents 59,812 shares of Common Stock issuable to Dr. Cooperstone upon exercise of outstanding options that are exercisable within 60 days of April 2, 2024, including 48,597 unvested shares which may be acquired through early option exercise.
- (14) Represents 1,395,093 shares of Common Stock issuable our current executive officers and directors upon exercise of outstanding options that are exercisable within 60 days of April 2, 2024, including 610,361 unvested shares which may be acquired through early option exercise.
- (15) Represents shares held by Adage Capital Partners, L.P. ("Adage Capital Partners"). Adage Capital Management, L.P. ("Adage Capital Management") has investment control over the securities held by Adage Capital Partners. Bob Atchinson and Phillip Gross are the managing members of Adage Capital Advisors, L.L.C., which is the managing member of Adage Capital Partners GP, L.L.C., which is the general partner of Adage Capital Management, and each such person or entity, as the case may be, has shared voting and/or investment power over the shares held by Adage Capital Partners and may be deemed the beneficial owner of such shares, and each such person or entity, as the case may be, disclaims beneficial ownership of such securities except to the extent of their respective pecuniary interest therein. The address of Adage Capital Partners is 200 Clarendon Street, 52nd Floor, Boston, Massachusetts 02116.
- (16) Represents shares held by Allostry Master Fund LP ("Allostry Master Fund"). Allostry Funds GP LLC ("Allostry Funds GP") may be deemed the beneficial owner of the shares held by Allostry Master Fund because it is the general partner of Allostry Master Fund. Allostry Investments LP ("Allostry Investments") may be deemed the beneficial owner of the shares held by Allostry Master Fund because it is the investment manager of Allostry Master Fund. Allostry Investments GP LLC (the "Investments GP") may be deemed

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the beneficial owner of the shares beneficially held by Allostery Investments because it is the general partner of Allostery Investments. Christopher Staral and David Modest may be deemed the beneficial owners of the shares beneficially held by each of Investments GP and Allostery Funds GP by virtue of their positions as managing members of Investments GP and Allostery Funds GP. The address of Allostery Master Fund is c/o Maples Corporate Services Limited, Ugland House, South Church Street, Grand Cayman KY1-1104, Cayman Islands.

- (17) Represents shares held by Braidwell Partners Master Fund LP (“Braidwell Partners”). The general partner of Braidwell Partners is Braidwell GP LLC (“Braidwell GP”), and the investment manager of Braidwell Partners is Braidwell LP. Braidwell Management LLC (“Braidwell Management”) is the managing member of both Braidwell GP and Braidwell LP. Alex Karnal and Brian Kreiter are the managing members of Braidwell Management and may be deemed to share voting and investment power with respect to the shares held by Braidwell Partners. Mr. Karnal and Mr. Kreiter disclaim beneficial ownership of the shares held by Braidwell Partners except to the extent of their pecuniary interests in such shares, if any. The address of Braidwell Partners is c/o Braidwell LP, One Harbor Point, 2200 Atlantic Street, Stamford, Connecticut 06902.
- (18) Represents shares held by Gray’s Creek Capital Partners Fund I, LP (“Gray’s Creek Capital Partners”). Gray’s Creek Capital Partners is managed by Gray’s Creek Capital Advisors, LLC (“Gray’s Creek Capital Advisors”) and Gray’s Creek Capital Partners, GP (“Gray’s Creek GP”). Jason R. Little and Gerrit B. Parker are the natural persons who have voting or investment control over the shares held by Gray’s Creek Capital Advisors and Gray’s Creek GP, and thus have voting or investment control over the shares being offered. The address of Gray’s Creek Capital Partners is 272 Post Road East, Westport, Connecticut 06880.
- (19) Represents shares held by Integrated Core Strategies (US) LLC and its affiliates, ICS Opportunities II LLC and ICS Opportunities, Ltd. These shares may be deemed to be beneficially owned by Millennium Management LLC, Millennium Group Management LLC and Mr. Englander and/or other investment managers that may be controlled by Millennium Group Management LLC (the managing member of Millennium Management LLC) and Mr. Englander (the sole voting trustee of the managing member of Millennium Group Management LLC). The foregoing should not be construed in and of itself as an admission by Millennium Management LLC, Millennium Group Management LLC or Mr. Englander as to the beneficial ownership of the securities held by such entities. The address for Integrated Core Strategies (US) LLC is c/o Millennium Management LLC, 399 Park Avenue, New York, New York 10022.
- (20) Represents shares held by Invus Public Equities, L.P. (“Invus PE”). Invus Public Equities Advisors, LLC (“Invus PE Advisors”) controls Invus PE, as its general partner and accordingly, may be deemed to beneficially own the shares held by Invus PE. The Geneva branch of Artal International S.C.A. (“Artal International”) controls Invus PE Advisors, as its managing member and accordingly, may be deemed to beneficially own the shares held by Invus PE. Artal International Management S.A. (“Artal International Management”), as the managing partner of Artal International, controls Artal International and accordingly, may be deemed to beneficially own the shares that Artal International may be deemed to beneficially own. Artal Group S.A. (“Artal Group”), as the sole stockholder of Artal International Management, controls Artal International Management and accordingly, may be deemed to beneficially own the shares that Artal International Management may be deemed to beneficially own. Westend S.A. (“Westend”), as the parent company of Artal Group, controls Artal Group and accordingly, may be deemed to beneficially own the shares that Artal Group may be deemed to beneficially own. Stichting Administratiekantoor Westend (the “Stichting”), as majority shareholder of Westend, controls Westend and accordingly, may be deemed to beneficially own the shares that Westend may be deemed to beneficially own. Mr. Amaury Wittouck, as the sole member of the board of the Stichting, controls the Stichting and accordingly, may be deemed to beneficially own the shares that the Stichting may be deemed to beneficially own. The address for Invus PE and Invus PE Advisors is 750 Lexington Avenue, 30th Floor, New York, NY 10022. The address for Artal International, Artal International Management, Artal Group, Westend and Mr. Wittouck is Valley Park, 44, Rue de la Vallée, L-2661, Luxembourg. The address for the Stichting is Claude Debussylaan, 46, 1082 MD Amsterdam, The Netherlands.
- (21) Represents shares held by Irving Investors, LLC. Jeremy Ableson is the natural person who has voting or investment control over the shares held by Irving Investors, LLC. The address of Irving Investors, LLC is 205 Detroit Street (4th Floor), Denver, Colorado 80206.
- (22) Represents shares held by Laurion Capital Master Fund Ltd. (“Laurion Capital Master Fund”). Laurion Capital Management LP (“Laurion Capital Management”), the investment manager of Laurion Capital Master Fund, has voting and investment power over the shares held by Laurion Capital Master Fund. Messrs. Benjamin A. Smith and Sheehan Maduraperuma are the managing members of Laurion Capital GP LLC (“Laurion Capital GP”), which is the general partner of Laurion Capital Management. Each of Laurion Capital Master Fund, Laurion Capital GP, Benjamin A. Smith and Sheehan Maduraperuma disclaims beneficial ownership over these shares. The address of Laurion Capital Master Fund is c/o Laurion Capital Management LP, 360 Madison Avenue, Suite 1900, New York, New York 10017.
- (23) Represents shares held by Novo Holdings A/S (“Novo”). Novo has the sole power to vote and dispose these shares, and no person or entity is deemed to have any beneficial ownership or reportable pecuniary interest in the shares held by Novo. The address for Novo is Tuborg Havnevej 19, DK-2900 Hellerup, Denmark.
- (24) Represents shares held by Octagon Investments Master Fund LP (“Octagon Master Fund”). Octagon Capital Advisors LP (“Octagon”) serves as the investment manager of Octagon Master Fund. Mr. Ting Jia is the managing member of Octagon. By virtue of these relationships, each of Octagon and Mr. Jia may be deemed to beneficially own the Company’s shares directly owned by Octagon Master Fund. The address of Octagon Master Fund is c/o Octagon 654 Madison Avenue 21st Floor, New York, New York 10065.
- (25) Represents shares held by RA Capital Healthcare Fund L.P. (“RA Capital Healthcare”). RA Capital Management, L.P. (“RA Capital Management”) is the investment manager for RA Capital Healthcare. The general partner of RA Capital Management is RA Capital Management GP, LLC (“RA Capital Management GP”), of which Peter Kolchinsky and Rajeev Shah are the managing members. Each of RA Capital Management, RA Capital Management GP, Mr. Kolchinsky and Mr. Shah may be deemed to have voting and investment power over the shares held by RA Capital Healthcare. RA Capital Management, RA Capital Management GP, Mr. Kolchinsky and Mr. Shah disclaim beneficial ownership of such shares, except to the extent of any pecuniary interest therein. The address of the persons and entities listed above is 200 Berkeley Street, 18th Floor, Boston, Massachusetts 02116.

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- (26) Represents (i) 81,812 shares held by Vestal Point Master Fund, LP (“Vestal Point Master Fund”) and (ii) 93,188 shares held by an account separately managed by Vestal Point Capital, LP (“Vestal Point Capital”). The sole general partner of Vestal Point Master Fund is Vestal Point Partners GP, LLC (“Vestal Point Partners GP”). The managing member of Vestal Point Partners GP is Ryan Wilder. The sole general partner of Vestal Point Capital is Vestal Point Capital, LLC. The managing member of Vestal Point Capital, LLC is Mr. Wilder. As a result, Mr. Wilder may be deemed to have voting and investment power over the shares held by Vestal Point Master Fund and the account separately managed by Vestal Point Capital. Mr. Wilder disclaims beneficial ownership of such shares, except to the extent of his pecuniary interest therein. The address of these entities and Mr. Wilder is c/o Vestal Point Capital, LP, 632 Broadway, Suite 602, New York, New York 10012.
- (27) Represents shares held by Woodline Master Fund LP (“Woodline Master Fund”). Woodline Partners LP (“Woodline Partners”) serves as the investment manager of Woodline Master Fund and may be deemed to be the beneficial owner of the shares. Woodline Partners disclaims any beneficial ownership of these shares. The address of Woodline Master Fund is 4 Embarcadero Center, Suite 3450, San Francisco, California 94111.
- (28) Represents shares held by Xantium Partners L.P. (“Xantium Partners”). Tudor Investment Corporation is the trading advisor that both directs investment and retains voting power for the shares held by Xantium Partners. The address for Xantium Partners is c/o Tudor Investment Corporation, 200 Elm Street, Stamford, Connecticut 06902.

DESCRIPTION OF CAPITAL STOCK

The following description summarizes certain important terms of our capital stock as of the date of this prospectus as specified in our certificate of incorporation and bylaws. Because the following description is only a summary, it does not contain all the information that may be important to you. For a complete description of the matters set forth in this section titled “*Description of Capital Stock*,” you should refer to the certificate of incorporation, the bylaws, and the Registration Rights Agreement, which are included as exhibits to the registration statement of which this prospectus is a part, and to the applicable provisions of Delaware law.

Authorized Capital Stock

Our authorized capital stock consists of 500,000,000 shares of Common Stock, \$0.0001 par value per share, and 10,000,000 shares of preferred stock, \$0.0001 par value per share. All of our outstanding shares of Common Stock are paid and nonassessable.

Common stock

Our Common Stock is listed on the Nasdaq Global Market under the symbol “LXEO.” The transfer agent and registrar for our Common Stock is Equiniti Trust Company, LLC. The transfer agent’s address is 6201 15th Avenue, Brooklyn, New York 11219.

Voting rights

Each holder of our Common Stock is entitled to one vote for each share on all matters submitted to a vote of the stockholders, including the election of directors. The affirmative vote of holders of at least 66-2/3% of the voting power of all of the then-outstanding shares of capital stock, voting as a single class, will be required to amend certain provisions of our amended and restated certificate of incorporation, including provisions relating to amending our amended and restated bylaws, the classified board, the size of our board, removal of directors, director liability, vacancies on our board, special meetings, stockholder notices, actions by written consent and exclusive forum.

Dividends

Subject to preferences that may be applicable to any then-outstanding preferred stock, holders of Common Stock are entitled to receive ratably those dividends, if any, as may be declared from time to time by the board of directors out of legally available funds.

Liquidation

In the event of our liquidation, dissolution or winding up, holders of Common Stock will be entitled to share ratably in the net assets legally available for distribution to stockholders after the payment of all of our debts and other liabilities and the satisfaction of any liquidation preference granted to the holders of any then-outstanding shares of preferred stock.

Rights and preferences

Holders of Common Stock have no preemptive, conversion or subscription rights and there are no redemption or sinking fund provisions applicable to the Common Stock. The rights, preferences and privileges of the holders of Common Stock are subject to, and may be adversely affected by, the right of the holders of shares of any series of preferred stock that we may designate in the future.

Registration rights of Certain Stockholders

Certain of our stockholders have registration rights under our amended and restated investors' rights agreement, or the Investors' Rights Agreement, between us and such stockholders. These stockholders (and certain of their permitted transferees) may request that we file registration statements under the Securities Act of 1933, as amended, and, upon such request and subject to minimum size and other conditions, we will be required to effect any such registration. We are generally obligated to bear the expenses, other than underwriting discounts and sales commissions, of these registrations. This summary does not purport to be complete and is qualified in its entirety by the provisions of the Investors' Rights Agreement, a copy of which has been filed as an exhibit to this Annual Report on Form 10-K.

Anti-takeover provisions

Section 203 of the Delaware General Corporation Law

We are subject to Section 203 of the Delaware General Corporation Law, which prohibits a Delaware corporation from engaging in any business combination with any interested stockholder for a period of three years after the date that such stockholder became an interested stockholder, with the following exceptions:

- before such date, the board of directors of the corporation approved either the business combination or the transaction that resulted in the stockholder becoming an interested stockholder;
- upon completion of the transaction that resulted in the stockholder becoming an interested stockholder, the interested stockholder owned at least 85% of the voting stock of the corporation outstanding at the time the transaction began, excluding for purposes of determining the voting stock outstanding, but not the outstanding voting stock owned by the interested stockholder, those shares owned (i) by persons who are directors and also officers and (ii) employee stock plans in which employee participants do not have the right to determine confidentially whether shares held subject to the plan will be tendered in a tender or exchange offer; or
- on or after such date, the business combination is approved by the board of directors and authorized at an annual or special meeting of the stockholders, and not by written consent, by the affirmative vote of at least 66-2/3% of the outstanding voting stock that is not owned by the interested stockholder.

In general, Section 203 defines a "business combination" to include the following:

- any merger or consolidation involving the corporation or any direct or indirect majority-owned subsidiary of the corporation and the interested stockholder;
- any sale, transfer, pledge or other disposition of 10% or more of the assets of the corporation involving the interested stockholder (in one transaction or a series of transactions);
- subject to certain exceptions, any transaction that results in the issuance or transfer by the corporation or by any direct or indirect majority-owned subsidiary of the corporation of any stock of the corporation or of such subsidiary to the interested stockholder;
- any transaction involving the corporation or any direct or indirect majority-owned subsidiary of the corporation that has the effect of increasing the proportionate share of the stock or any class or series of the corporation beneficially owned by the interested stockholder; or
- the receipt by the interested stockholder of the benefit of any loans, advances, guarantees, pledges or other financial benefits by or through the corporation.

In general, Section 203 defines an "interested stockholder" as an entity or person who, together with the person's affiliates and associates, beneficially owns, or within three years prior to the time of determination of interested stockholder status did own, 15% or more of the outstanding voting stock of the corporation.

Amended and restated certificate of incorporation and amended and restated bylaws

Our amended and restated certificate of incorporation provides for our board of directors to be divided into three classes with staggered three-year terms. Only one class of directors will be elected at each annual meeting of our stockholders, with the other classes continuing for the remainder of their respective three-year terms. Because our stockholders do not have cumulative voting rights, stockholders holding a majority of the shares of Common Stock outstanding will be able to elect all of our directors. Our amended and restated certificate and our amended and restated bylaws also provides that directors may be removed by the stockholders only for cause upon the vote of 66-2/3% or more of our outstanding Common Stock. Furthermore, the authorized number of directors may be changed only by resolution of the board of directors, and vacancies and newly created directorships on the board of directors may, except as otherwise required by law or determined by the board, only be filled by a majority vote of the directors then serving on the board, even though less than a quorum.

Under our amended and restated certificate of incorporation and amended and restated bylaws our stockholders do not have cumulative voting rights. Because of this, the holders of a majority of the shares of Common Stock entitled to vote in any election of directors can elect all of the directors standing for election, if they should so choose.

Our amended and restated certificate and amended and restated bylaws also provide that all stockholder actions must be effected at a duly called meeting of stockholders and will eliminate the right of stockholders to act by written consent without a meeting. Our amended and restated bylaws also provide that only our Chairman of the board, Chief Executive Officer or the board of directors pursuant to a resolution adopted by a majority of the total number of authorized directors may call a special meeting of stockholders.

Our amended and restated bylaws also provide that stockholders seeking to present proposals before a meeting of stockholders to nominate candidates for election as directors at a meeting of stockholders must provide timely advance notice in writing, and will specify requirements as to the form and content of a stockholder's notice.

Our amended and restated certificate and amended and restated bylaws provide that our stockholders cannot amend many of the provisions described above except by a vote of 66-2/3% or more of our outstanding Common Stock.

The combination of these provisions makes it more difficult for our existing stockholders to replace our board of directors as well as for another party to obtain control of us by replacing our board of directors. Since our board of directors has the power to retain and discharge our officers, these provisions could also make it more difficult for existing stockholders or another party to effect a change in management. In addition, the authorization of undesignated preferred stock makes it possible for our board of directors to issue preferred stock with voting or other rights or preferences that could impede the success of any attempt to change our control.

These provisions are intended to enhance the likelihood of continued stability in the composition of our board of directors and its policies and to discourage coercive takeover practices and inadequate takeover bids. These provisions are also designed to reduce our vulnerability to hostile takeovers and to discourage certain tactics that may be used in proxy fights. However, such provisions could have the effect of discouraging others from making tender offers for our shares and may have the effect of delaying changes in our control or management. As a consequence, these provisions may also inhibit fluctuations in the market price of our stock that could result from actual or rumored takeover attempts. We believe that the benefits of these provisions, including increased protection of our potential ability to negotiate with the proponent of an unfriendly or unsolicited proposal to acquire or restructure our company, outweigh the disadvantages of discouraging takeover proposals, because negotiation of takeover proposals could result in an improvement of their terms.

Choice of forum

Our amended and restated certificate of incorporation provides that the Court of Chancery of the state of Delaware will be the exclusive forum for the following types of actions or proceedings under Delaware statutory or common law:

- any derivative action or proceeding brought on our behalf;
- any action asserting a breach of fiduciary duty;
- any action asserting a claim against us arising pursuant to the Delaware General Corporation Law, our restated certificate, or our amended and restated bylaws; or
- any action asserting a claim against us that is governed by the internal affairs doctrine.

The provision would not apply to suits brought to enforce a duty or liability created by the Exchange Act. Furthermore, Section 22 of the Securities Act creates concurrent jurisdiction for federal and state courts over all such Securities Act actions. Accordingly, both state and federal courts have jurisdiction to entertain such claims. To prevent having to litigate claims in multiple jurisdictions and the threat of inconsistent or contrary rulings by different courts, among other considerations, our amended and restated certificate of incorporation will also provide that unless we consent in writing to the selection of an alternative forum, the federal district courts of the United States shall be the exclusive forum for the resolution of any complaint asserting a cause of action arising under the Securities Act.

While the Delaware courts have determined that such choice of forum provisions are facially valid, a stockholder may nevertheless seek to bring a claim in a venue other than those designated in the exclusive forum provisions. In such instance, we would expect to vigorously assert the validity and enforceability of the exclusive forum provisions of our restated certificate of incorporation. This may require significant additional costs associated with resolving such action in other jurisdictions and there can be no assurance that the provisions will be enforced by a court in those other jurisdictions.

These exclusive forum provisions may limit a stockholder's ability to bring a claim in a judicial forum that it finds favorable for disputes with us or our directors, officers, or other employees, which may discourage lawsuits against us and our directors, officers and other employees. If a court were to find either exclusive-forum provision in our amended and restated certificate of incorporation to be inapplicable or unenforceable in an action, we may incur further significant additional costs associated with resolving the dispute in other jurisdictions, all of which could seriously harm our business.

Our amended and restated certificate of incorporation further provides that the federal district courts of the United States of America will be the exclusive forum for resolving any complaint asserting a cause of action arising under the Securities Act, subject to and contingent upon a final adjudication in the State of Delaware of the enforceability of such exclusive forum provision.

Transfer agent and registrar

The transfer agent and registrar for our Common Stock is Equiniti Trust Company, LLC. The transfer agent's address is 6201 15th Avenue, Brooklyn, New York 11219.

Listing

Our Common Stock is listed on the Nasdaq Global Market under the trading symbol "LXEO."

Shares eligible for future sale

Prior to our IPO, no public market existed for our Common Stock. Future sales of our Common Stock in the public market, or the availability of such shares for sale in the public market, could adversely affect market prices

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prevailing from time to time. As discussed further below, a limited number of shares is currently available for sale due to contractual and legal restrictions on resale. Nevertheless, sales of our Common Stock in the public market after such restrictions lapse, or the perception that those sales may occur, could adversely affect the prevailing market price at such time and our ability to raise equity capital in the future.

Rule 144

In general, non-affiliate persons who have beneficially owned restricted shares of our Common Stock for at least six months, and any affiliate of the company who owns either restricted or unrestricted shares of our Common Stock, are entitled to sell their securities without registration with the SEC under an exemption from registration provided by Rule 144 under the Securities Act.

Non-affiliates

Any person who is not deemed to have been one of our affiliates at the time of, or at any time during the three months preceding, a sale may sell an unlimited number of restricted securities under Rule 144 if:

- the restricted securities have been held for at least six months, including the holding period of any prior owner other than one of our affiliates (subject to certain exceptions);
- we have been subject to the Exchange Act periodic reporting requirements for at least 90 days before the sale; and
- we are current in our Exchange Act reporting at the time of sale.

Any person who is not deemed to have been an affiliate of ours at the time of, or at any time during the three months preceding, a sale and has held the restricted securities for at least one year, including the holding period of any prior owner other than one of our affiliates, will be entitled to sell an unlimited number of restricted securities without regard to the length of time we have been subject to Exchange Act periodic reporting or whether we are current in our Exchange Act reporting. Non-affiliate resales are not subject to the manner of sale, volume limitation or notice filing provisions of Rule 144.

Affiliates

Persons seeking to sell restricted securities who are our affiliates at the time of, or any time during the three months preceding, a sale, would be subject to the restrictions described above. They are also subject to additional restrictions, by which such person would be required to comply with the manner of sale and notice provisions of Rule 144 and would be entitled to sell within any three-month period only that number of securities that does not exceed the greater of either of the following:

- 1% of the number of shares of our Common Stock then outstanding, which will equal approximately 329,478 shares immediately after the completion of the Private Placement, and based on the number of shares outstanding as of December 31, 2023; or
- the average weekly trading volume of our Common Stock on the stock exchange on which our shares are listed during the four calendar weeks preceding the filing of a notice on Form 144 with respect to the sale.

Additionally, persons who are our affiliates at the time of, or any time during the three months preceding, a sale may sell unrestricted securities under the requirements of Rule 144 described above, without regard to the six-month holding period of Rule 144, which does not apply to sales of unrestricted securities.

Rule 701

Rule 701 under the Securities Act, as in effect on the date of this prospectus, permits resales of shares in reliance upon Rule 144 but without compliance with certain restrictions of Rule 144, including the holding period

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requirement. Most of our employees, executive officers or directors who purchased shares under a written compensatory plan or contract may be entitled to rely on the resale provisions of Rule 701, but all holders of Rule 701 shares are required to wait until 90 days after the date of this prospectus before selling their shares. However, substantially all Rule 701 shares are subject to lock-up agreements as described below and will become eligible for sale upon the expiration of the restrictions set forth in those agreements.

Lock-up agreements

We, our executive officers and directors and substantially all of the holders of our Common Stock outstanding immediately prior to our IPO entered into lock-up agreements with the underwriters in our IPO or otherwise agreed, subject to limited exceptions, that we and they will not, offer, pledge, announce the intention to sell, sell, contract to sell, sell any option or contract to purchase, purchase any option or contract to sell, grant any option, right or warrant to purchase or otherwise dispose of, directly or indirectly, or enter into any swap or other agreement that transfers, in whole or in part, any of the economic consequences of ownership of our Common Stock, or any securities convertible into, or exchangeable for or that represent the right to receive shares of our Common Stock, without the prior written consent of J.P. Morgan Securities LLC and Leerink Partners LLC (the “Underwriters”) for a period of 180 days from November 2, 2023. In connection with the Private Placement, we and the Underwriters agreed to extend the lock-up agreement applicable to the Company for a period of 90 days from the Closing Date.

In addition to the restrictions contained in the lock-up agreements entered into in connection with our IPO, we entered into an agreement with the holders of our preferred stock that contains market stand-off provisions imposing restrictions on the ability of such security holders to sell or otherwise transfer or dispose of any registrable securities for a period of 180 days following November 2, 2023.

In connection with the Private Placement, we entered into lock-up agreements with our directors and executive officers. Subject to certain exceptions, our directors and executive officers agreed to a lock-up on their respective shares of Common Stock during the period beginning on March 11, 2024, and ending 90 days after the Closing Date.

Registration rights

The holders of 20,325,492 shares of our Common Stock (including 6,974,248 Shares offered by the Selling Stockholders hereunder), or their transferees, are entitled to specified rights with respect to the registration of their registrable shares under the Securities Act, subject to certain limitations and the expiration, waiver or termination of the lock-up agreements. Registration of these shares under the Securities Act would result in the shares becoming freely tradable without restriction under the Securities Act immediately upon effectiveness of the registration. See “*Description of Capital Stock—Registration Rights*” for additional information.

In connection with the Purchase Agreement, we entered into the Registration Rights Agreement with the Selling Stockholders, pursuant to which, we agreed that within 30 days after the closing of the Private Placement, we will file with the SEC (at our sole cost and expense) a registration statement registering the resale of the shares of Common Stock sold in the Private Placement, and we shall use best efforts to have the registration statement declared effective as promptly as possible after the filing thereof, subject to the provisions set forth in the Registration Rights Agreement.

SECURITIES ACT RESTRICTIONS ON RESALE OF SECURITIES

Rule 144

A person who has beneficially owned restricted shares of Common Stock for at least six months would be entitled to sell their securities provided that (i) such person is not deemed to have been one of our affiliates at the time of, or at any time during the three months preceding, a sale and (ii) we are subject to the Exchange Act periodic reporting requirements for at least three months before the sale. Persons who have beneficially owned restricted shares of Common Stock for at least six months but who are our affiliates at the time of, or any time during the three months preceding, a sale, would be subject to additional restrictions, by which such person would be entitled to sell within any three-month period a number of securities that does not exceed the greater of:

- 1% of the then outstanding equity shares of the same class; and
- the average weekly trading volume of Common Stock, as applicable, during the four calendar weeks preceding the date on which notice of the sale is filed with the SEC.

Sales by affiliates of Lexeo under Rule 144 are also subject to certain requirements relating to manner of sale, notice and the availability of current public information about Lexeo.

**MATERIAL U.S. FEDERAL INCOME TAX CONSIDERATIONS
FOR NON-U.S. HOLDERS OF OUR COMMON STOCK**

The following is a summary of material U.S. federal income tax considerations of the ownership and disposition of our Common Stock acquired in this offering by a “non-U.S. holder” (as defined below) but does not purport to be a complete analysis of all the potential tax considerations relating thereto. This summary is based on the provisions of the Internal Revenue Code of 1986, as amended, or the Code, Treasury Regulations promulgated thereunder, administrative rulings and judicial decisions, all as of the date hereof. These authorities may be changed, possibly retroactively, so as to result in U.S. federal income tax considerations different from those set forth below. We have not sought, and do not intend to seek, any ruling from the Internal Revenue Service, or the IRS, with respect to the statements made and the conclusions reached in the following summary, and there can be no assurance that the IRS or a court will agree with such statements and conclusions.

This summary also does not address the tax considerations arising under the laws of any U.S. state or local or non-U.S. jurisdiction or under U.S. federal gift and estate tax rules, or the effect, if any, of the Medicare contribution tax on net investment income. In addition, this discussion does not address tax considerations applicable to an investor’s particular circumstances or to investors that may be subject to special tax rules, including, without limitation:

- banks, insurance companies, regulated investment companies, real estate investment trusts or other financial institutions;
- persons subject to the alternative minimum tax;
- tax-exempt or governmental organizations;
- pension plans and tax-qualified retirement plans;
- controlled foreign corporations, passive foreign investment companies and corporations that accumulate earnings to avoid U.S. federal income tax;
- entities or arrangements classified as partnerships for U.S. federal income tax purposes or other pass-through entities (or investors in such entities or arrangements);
- brokers or dealers in securities or currencies;
- traders in securities that elect to use a mark-to-market method of tax accounting for their securities holdings;
- persons who own, or are deemed to own, more than five percent of our capital stock (except to the extent specifically set forth below);
- certain former citizens or long-term residents of the United States;
- persons who hold our Common Stock as a position in a hedging transaction, “straddle,” “conversion transaction,” or other risk reduction transaction;
- persons who hold or receive our Common Stock pursuant to the exercise of any option or otherwise as compensation;
- persons who do not hold our Common Stock as a capital asset within the meaning of Section 1221 of the Code (generally, property held for investment);
- persons deemed to sell our Common Stock under the constructive sale provisions of the Code; or
- persons subject to special tax accounting rules as a result of any item of gross income with respect to our Common Stock being taken into account in an “applicable financial statement” as defined in Section 451(b) of the Code.

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In addition, if a partnership (or other entity or arrangement classified as a partnership for U.S. federal income tax purposes) holds our Common Stock, the tax treatment of a partner in the partnership generally will depend on the status of the partner and upon the activities of the partnership. A partner in a partnership that will hold our Common Stock should consult his, her or its own tax advisor regarding the tax considerations of the ownership and disposition of our Common Stock through a partnership.

You are urged to consult your tax advisor with respect to the application of the U.S. federal income tax laws to your particular situation, as well as any tax considerations of the ownership and disposition of our Common Stock arising under the U.S. federal gift or estate tax rules or under the laws of any U.S. state or local, non-U.S. or other taxing jurisdiction or under any applicable income tax treaty.

Non-U.S. Holder Defined

For purposes of this discussion, you are a “non-U.S. holder” if you are a beneficial owner of our Common Stock that, for U.S. federal income tax purposes, is neither a partnership nor:

- an individual who is a citizen or resident of the United States;
- a corporation or other entity taxable as a corporation created or organized in the United States or under the laws of the United States or any political subdivision thereof, or otherwise treated as such for U.S. federal income tax purposes;
- an estate whose income is subject to U.S. federal income tax regardless of its source; or
- a trust (x) whose administration is subject to the primary supervision of a U.S. court and that has one or more U.S. persons who have the authority to control all substantial decisions of the trust or (y) that has made a valid election under applicable Treasury Regulations to be treated as a U.S. person.

Dividends

As described in the section titled “*Dividend Policy*,” we have never declared or paid cash dividends on our Common Stock, and we do not anticipate paying any dividends on our Common Stock following the completion of this offering. However, if we do make distributions on our Common Stock, those payments will constitute dividends for U.S. federal income tax purposes to the extent paid from our current or accumulated earnings and profits, as determined under U.S. federal income tax principles. To the extent those distributions exceed both our current and our accumulated earnings and profits, the excess will constitute a return of capital and will first reduce your basis in our Common Stock, but not below zero, and then will be treated as gain from the sale of stock as described below under “—*Gain on Disposition of Common Stock*.”

Subject to the discussions below regarding effectively connected income, backup withholding and FATCA, any dividend paid to you generally will be subject to U.S. federal withholding tax either at a rate of 30% of the gross amount of the dividend or such lower rate as may be specified by an applicable income tax treaty between the United States and your country of residence. In order to receive a reduced treaty rate, you must provide us or the applicable paying agent with an IRS Form W-8BEN or Form W-8BEN-E or other appropriate version of IRS Form W-8 certifying qualification for the reduced rate. Under applicable Treasury Regulations, we may withhold up to 30% of the gross amount of the entire distribution even if the amount constituting a dividend, as described above, is less than the gross amount. You may obtain a refund of any excess amounts withheld by filing an appropriate claim for refund with the IRS. If you hold our Common Stock through a financial institution or other agent acting on your behalf, you will be required to provide appropriate documentation to the agent, which then will be required to provide certification to us or our paying agent, either directly or through other intermediaries.

Dividends received by you that are treated as effectively connected with your conduct of a U.S. trade or business (and, if required by an applicable income tax treaty, that are attributable to a permanent establishment or fixed base maintained by you in the United States) are generally exempt from the 30% U.S. federal withholding

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tax, subject to the discussions below regarding backup withholding and FATCA. In order to obtain this exemption, you must provide us with a properly executed IRS Form W-8ECI or other applicable IRS Form W-8 properly certifying such exemption. Such effectively connected dividends, although not subject to U.S. federal withholding tax, generally are taxed at the U.S. federal income tax rates applicable to U.S. persons, net of certain deductions and credits. In addition, if you are a corporate non-U.S. holder, dividends you receive that are effectively connected with your conduct of a U.S. trade or business may also be subject to a branch profits tax at a rate of 30% or such lower rate as may be specified by an applicable income tax treaty between the United States and your country of residence. You should consult your tax advisor regarding the tax consequences of the ownership and disposition of our Common Stock, including the application of any applicable tax treaties that may provide for different rules.

Gain on Disposition of Common Stock

Subject to the discussions below regarding backup withholding and FATCA, you generally will not be required to pay U.S. federal income tax on any gain realized upon the sale or other disposition of our Common Stock unless:

- the gain is effectively connected with your conduct of a U.S. trade or business (and, if an applicable income tax treaty so provides, the gain is attributable to a permanent establishment or fixed base maintained by you in the United States);
- you are an individual who is present in the United States for a period or periods aggregating 183 days or more during the calendar year in which the sale or disposition occurs and certain other conditions are met; or
- our Common Stock constitutes a United States real property interest by reason of our status as a “United States real property holding corporation,” or a USRPHC, for U.S. federal income tax purposes at any time within the shorter of the five-year period preceding your disposition of, or your holding period for, our Common Stock.

We believe that we are not currently and will not become a USRPHC for U.S. federal income tax purposes, and the remainder of this discussion so assumes. However, because the determination of whether we are a USRPHC depends on the fair market value of our U.S. real property interests relative to the fair market value of our U.S. and worldwide real property interests plus our other assets used or held for use in a trade or business, there can be no assurance that we will not become a USRPHC in the future. Even if we become a USRPHC, however, as long as our Common Stock is regularly traded on an established securities market, your Common Stock will be treated as U.S. real property interests only if you actually (directly or indirectly) or constructively hold more than five percent of our regularly traded Common Stock at any time during the shorter of the five-year period preceding your disposition of, or your holding period for, our Common Stock.

If you are a non-U.S. holder described in the first bullet above, you generally will be required to pay tax on the gain derived from the sale (net of certain deductions and credits) under U.S. federal income tax rates applicable to U.S. persons, and a corporate non-U.S. holder described in the first bullet above also may be subject to the branch profits tax at a 30% rate, or such lower rate as may be specified by an applicable income tax treaty. If you are an individual non-U.S. holder described in the second bullet above, you will be subject to tax at 30% (or such lower rate specified by an applicable income tax treaty) on the gain derived from the sale, which gain may be offset by U.S. source capital losses for the year, provided you have timely filed U.S. federal income tax returns with respect to such losses. You should consult your tax advisor regarding any applicable income tax or other treaties that may provide for different rules.

Backup Withholding and Information Reporting

Generally, we must report annually to the IRS the amount of dividends paid to you, your name and address and the amount of tax withheld, if any. A similar report will be sent to you. Pursuant to applicable income tax

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treaties or other agreements, the IRS may make these reports available to tax authorities in your country of residence.

Payments of dividends on or of proceeds from the disposition of our Common Stock made to you may be subject to backup withholding at the applicable statutory rate unless you establish an exemption, for example, by properly certifying your non-U.S. status on a properly completed IRS Form W-8BEN or W-8BEN-E or another appropriate version of IRS Form W-8, as applicable. Notwithstanding the foregoing, backup withholding and information reporting may apply if either we or our paying agent has actual knowledge, or reason to know, that you are a U.S. person.

Backup withholding is not an additional tax; rather, the U.S. federal income tax liability of persons subject to backup withholding will be reduced by the amount of tax withheld. If withholding results in an overpayment of taxes, a refund or credit may generally be obtained from the IRS, provided that the required information is furnished to the IRS in a timely manner.

Additional Withholding Requirements under the Foreign Account Tax Compliance Act

Subject to the following paragraph, the Foreign Account Tax Compliance Act and the Treasury Regulations and other official IRS guidance issued thereunder (collectively, "FATCA") generally imposes a U.S. federal withholding tax of 30% on dividends on, and the gross proceeds from a sale or other disposition of, our Common Stock, paid to a "foreign financial institution" (as specially defined under these rules), unless such institution enters into an agreement with the U.S. government to, among other things, withhold on certain payments and to collect and provide to the U.S. tax authorities substantial information regarding the U.S. account holders of such institution (which includes certain equity and debt holders of such institution, as well as certain account holders that are non-U.S. entities with U.S. owners) or otherwise establishes an exemption. Subject to the following paragraph, FATCA also generally imposes a U.S. federal withholding tax of 30% on dividends on, and the gross proceeds from a sale or other disposition of, our Common Stock paid to a "non-financial foreign entity" (as specially defined under these rules) unless such entity provides the withholding agent with a certification identifying the substantial direct and indirect U.S. owners of the entity, certifies that it does not have any substantial U.S. owners, or otherwise establishes an exemption. The withholding tax will apply regardless of whether the payment otherwise would be exempt from the U.S. nonresident withholding tax described above and backup withholding, including under the exemptions described above. Under certain circumstances, you might be eligible for refunds or credits of such taxes. An intergovernmental agreement between the United States and your country of residence may modify the requirements described in this section. You should consult with your own tax advisors regarding the application of FATCA to your ownership and disposition of our Common Stock.

The U.S. Treasury Department has issued proposed regulations that, if finalized in their present form, would eliminate FATCA withholding on gross proceeds of the sale or other disposition of our Common Stock (but not on payments of dividends). The preamble of such proposed regulations states that they may be relied upon by taxpayers until final regulations are issued or until such proposed regulations are rescinded.

The preceding discussion of U.S. federal income tax considerations is for general informational purposes only. It is not tax advice to investors in their particular circumstances. You should consult your own tax advisor regarding the particular U.S. federal, state and local and non-U.S. tax considerations of owning and disposing of our Common Stock, including the consequences of any proposed change in applicable laws.

PLAN OF DISTRIBUTION

The Selling Stockholders, which as used herein includes donees, pledgees, transferees or other successors-in-interest selling shares of Common Stock or interests in shares of Common Stock received after the date of this prospectus from a Selling Stockholder as a gift, pledge, partnership distribution or other transfer, may, from time to time, sell, transfer, distribute in kind for no consideration or otherwise dispose of any or all of their shares of Common Stock or interests in shares of Common Stock on any stock exchange, market or trading facility on which the shares are traded or in private transactions. These dispositions may be at fixed prices, at prevailing market prices at the time of sale, at prices related to the prevailing market price, at varying prices determined at the time of sale, or at negotiated prices.

The Selling Stockholders may use any one or more of the following methods when disposing of shares or interests therein:

- ordinary brokerage transactions and transactions in which the broker-dealer solicits purchasers;
- block trades in which the broker-dealer will attempt to sell the shares as agent, but may position and resell a portion of the block as principal to facilitate the transaction;
- purchases by a broker-dealer as principal and resale by the broker-dealer for its account;
- an exchange distribution in accordance with the rules of the applicable exchange;
- privately negotiated transactions;
- short sales effected after the date the registration statement of which this prospectus is a part is declared effective by the SEC;
- through the writing or settlement of options or other hedging transactions, whether through an options exchange or otherwise;
- broker-dealers may agree with the Selling Stockholders to sell a specified number of such shares at a stipulated price per share;
- through the distribution of the securities by any Selling Stockholder to its partners, members or stockholders;
- by pledge to secured debts and other obligations or any transfer upon the foreclosure under such pledges;
- directly to one or more purchasers;
- through delayed delivery requirements;
- a combination of any such methods of sale; and
- any other method permitted by applicable law.

The Selling Stockholders may, from time to time, pledge or grant a security interest in some or all of the shares of Common Stock owned by them and, if they default in the performance of their secured obligations, the pledgees or secured parties may offer and sell the shares of Common Stock, from time to time, under this prospectus, or under an amendment to this prospectus under Rule 424(b)(3) or other applicable provision of the Securities Act of 1933, as amended (the “**Securities Act**”), amending the list of Selling Stockholders to include the pledgee, transferee or other successors in interest as Selling Stockholders under this prospectus. The Selling Stockholders also may transfer the shares of Common Stock in other circumstances, in which case the transferees, pledgees or other successors in interest will be the Selling Stockholders for purposes of this prospectus.

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In connection with the sale of our Common Stock or interests therein, the Selling Stockholders may enter into hedging transactions with broker-dealers or other financial institutions, which may in turn engage in short sales of the Common Stock in the course of hedging the positions they assume. The Selling Stockholders may also sell shares of our Common Stock short and deliver these securities to close out their short positions, or loan or pledge the Common Stock to broker-dealers that in turn may sell these securities. The Selling Stockholders may also enter into option or other transactions with broker-dealers or other financial institutions or the creation of one or more derivative securities which require the delivery to such broker-dealer or other financial institution of shares offered by this prospectus, which shares such broker-dealer or other financial institution may resell pursuant to this prospectus (as supplemented or amended to reflect such transaction).

The aggregate proceeds to the Selling Stockholders from the sale of the Common Stock offered by them will be the purchase price of the Common Stock less discounts or commissions, if any. Each of the Selling Stockholders reserves the right to accept and, together with their agents from time to time, to reject, in whole or in part, any proposed purchase of Common Stock to be made directly or through agents. The Company will not receive any of the proceeds from this offering.

The Selling Stockholders also may resell all or a portion of the shares in open market transactions in reliance upon Rule 144 under the Securities Act, provided that they meet the criteria and conform to the requirements of that rule, or another available exemption from the registration requirements of the Securities Act.

The Selling Stockholders and any underwriters, broker-dealers or agents that participate in the sale of the Common Stock or interests therein may be “underwriters” within the meaning of Section 2(a)(11) of the Securities Act (it being understood that the Selling Stockholders shall not be deemed to be underwriters solely as a result of their participation in this offering). Any discounts, commissions, concessions or profit they earn on any resale of the shares may be underwriting discounts and commissions under the Securities Act. Selling stockholders who are “underwriters” within the meaning of Section 2(a)(11) of the Securities Act will be subject to the prospectus delivery requirements of the Securities Act.

To the extent required, the shares of our Common Stock to be sold, the names of the Selling Stockholders, the respective purchase prices and public offering prices, the names of any agents, dealer or underwriter, and any applicable commissions or discounts with respect to a particular offer will be set forth in an accompanying prospectus supplement or, if appropriate, a post-effective amendment to the registration statement that includes this prospectus.

In order to comply with the securities laws of some states, if applicable, the Common Stock may be sold in these jurisdictions only through registered or licensed brokers or dealers. In addition, in some states the Common Stock may not be sold unless it has been registered or qualified for sale or an exemption from registration or qualification requirements is available and is complied with.

We have advised the Selling Stockholders that the anti-manipulation rules of Regulation M under the Securities Exchange Act of 1934, as amended, may apply to sales of shares in the market and to the activities of the Selling Stockholders and their affiliates. In addition, to the extent applicable, we will make copies of this prospectus (as it may be supplemented or amended from time to time) available to the Selling Stockholders for the purpose of satisfying the prospectus delivery requirements of the Securities Act. The Selling Stockholders may indemnify any broker-dealer that participates in transactions involving the sale of the shares against certain liabilities, including liabilities arising under the Securities Act.

We have agreed to indemnify the Selling Stockholders against liabilities, including liabilities under the Securities Act and state securities laws, relating to the registration of the shares offered by this prospectus.

We have agreed with the Selling Stockholders to use commercially reasonable efforts to cause the registration statement of which this prospectus constitutes a part to become effective and to remain continuously

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effective until the earlier of (1) such time as all of the shares covered by this prospectus have been disposed of pursuant to and in accordance with such registration statement or (2) the date that all the shares covered by this prospectus cease to be Registrable Securities.

We will bear all costs, expenses and fees in connection with the registration of the Selling Stockholders' securities, which will total approximately \$139,000.

LEGAL MATTERS

The validity of the Securities offered hereby has been passed upon for us by Wilson Sonsini Goodrich & Rosati, Professional Corporation, New York, New York, which has acted as our counsel in connection with this offering.

EXPERTS

The financial statements of Lexeo Therapeutics, Inc. as of December 31, 2023 and 2022, and for the years then ended have been included herein in reliance upon the report of KPMG LLP, independent registered public accounting firm, appearing elsewhere herein, and upon the authority of said firm as experts in accounting and auditing.

WHERE YOU CAN FIND ADDITIONAL INFORMATION

We have filed with the SEC a registration statement on Form S-1 under the Securities Act that registers the shares of our Common Stock to be offered by this prospectus. This prospectus constitutes only a part of the registration statement. Some items are contained in exhibits to the registration statement as permitted by the rules and regulations of the SEC. For further information with respect to us and our securities, we refer you to the registration statement, including the exhibits filed as a part of the registration statement. Statements contained in this prospectus concerning the contents of any contract or document referred to are not necessarily complete. If a contract or document has been filed as an exhibit to the registration statement, please see the copy of the contract or document that has been filed. Each statement in this prospectus relating to a contract or document filed as an exhibit is qualified in all respects by the filed exhibit.

We file annual, quarterly and current reports, proxy statements and other information with the SEC. The SEC maintains an Internet website at www.sec.gov that contains reports, proxy and information statements and other information about issuers, like us, that file electronically with the SEC. We also maintain a website at www.lexeotx.com. We make available, free of charge, on our investor relations website at ir.lexeotx.com, our Annual Reports on Form 10-K, Quarterly Reports on Form 10-Q, Current Reports on Form 8-K and amendments to these reports as soon as reasonably practicable after electronically filing or furnishing those reports to the SEC. Information contained on our website is not a part of or incorporated by reference into this prospectus and the inclusion of our website and investor relations website addresses in this prospectus is an inactive textual reference only.

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**LEXEO THERAPEUTICS, INC.
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KPMG LLP
345 Park Avenue
New York, NY 10154-0102

Report of Independent Registered Public Accounting Firm

To the Stockholders and Board of Directors

Lexeo Therapeutics, Inc.:

Opinion on the Consolidated Financial Statements

We have audited the accompanying consolidated balance sheets of Lexeo Therapeutics, Inc. and subsidiary (the Company) as of December 31, 2023 and 2022, the related consolidated statements of operations and comprehensive loss, convertible preferred stock and stockholders' equity (deficit), and cash flows for the years then ended, and the related notes (collectively, the consolidated financial statements). In our opinion, the consolidated financial statements present fairly, in all material respects, the financial position of the Company as of December 31, 2023 and 2022, and the results of its operations and its cash flows for the years then ended, in conformity with U.S. generally accepted accounting principles.

Basis for Opinion

These consolidated financial statements are the responsibility of the Company's management. Our responsibility is to express an opinion on these consolidated financial statements based on our audits. We are a public accounting firm registered with the PCAOB and are required to be independent with respect to the Company in accordance with the U.S. federal securities laws and the applicable rules and regulations of the Securities and Exchange Commission and the PCAOB.

We conducted our audits in accordance with the standards of the PCAOB. Those standards require that we plan and perform the audit to obtain reasonable assurance about whether the consolidated financial statements are free of material misstatement, whether due to error or fraud. Our audits included performing procedures to assess the risks of material misstatement of the consolidated financial statements, whether due to error or fraud, and performing procedures that respond to those risks. Such procedures included examining, on a test basis, evidence regarding the amounts and disclosures in the consolidated financial statements. Our audits also included evaluating the accounting principles used and significant estimates made by management, as well as evaluating the overall presentation of the consolidated financial statements. We believe that our audits provide a reasonable basis for our opinion.

KPMG LLP

We have served as the Company's auditor since 2021.

New York, New York
March 11, 2024

KPMG LLP, a Delaware limited liability partnership and a member firm of the KPMG global organization of independent member firms affiliated with KPMG International Limited, a private English company limited by guarantee.

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Lexeo Therapeutics, Inc. Consolidated Balance Sheets

(In thousands, except share and per share amounts)

	December 31, 2023	December 31, 2022
Assets		
Current assets:		
Cash and cash equivalents	\$ 121,466	\$ 77,335
Prepaid expenses	2,825	2,342
Other current assets	3	371
Total current assets	124,294	80,048
Restricted cash	3,252	3,253
Property and equipment, net	1,056	998
Lease right-of-use assets—finance, net	1,763	2,069
Lease right-of-use assets—operating	9,442	10,708
Total assets	<u>\$ 139,807</u>	<u>\$ 97,076</u>
Liabilities, Convertible Preferred Stock and Stockholders' Equity (Deficit)		
Current liabilities:		
Accounts payable	\$ 3,794	\$ 2,678
Taxes payable	157	506
Accrued expenses and other current liabilities	7,873	6,420
Accrued bonuses	2,810	1,911
Current portion of lease liabilities—finance	518	547
Current portion of lease liabilities—operating	2,087	2,287
Total current liabilities	17,239	14,349
Non-current liabilities		
Non-current portion of lease liabilities—finance	1,247	1,648
Non-current portion of lease liabilities—operating	7,786	9,000
Total liabilities	26,272	24,997
Commitments and contingencies (Note 12)		
Series A convertible preferred stock, no par value; no shares authorized, issued, and outstanding as of December 31, 2023; \$0.0001 par value; 85,495,722 shares authorized, issued, and outstanding as of December 31, 2022; aggregate liquidation value of \$85,496 as of December 31, 2022	—	85,268
Series B convertible preferred stock, no par value; no shares authorized, issued, and outstanding as of December 31, 2023; \$0.0001 par value; 58,157,824 shares authorized as of December 31, 2022; 58,157,823 shares issued and outstanding as of December 31, 2022; aggregate liquidation value of \$100,060 as of December 31, 2022	—	99,765
Stockholders' equity (deficit):		
Common stock, \$0.0001 par value, 500,000,000 shares authorized as of December 31, 2023; 26,668,485 shares issued and 26,646,378 shares outstanding as of December 31, 2023; 18,125,051 shares authorized as of December 31, 2022; 1,648,734 shares issued and 1,607,185 shares outstanding as of December 31, 2022	3	—
Additional paid-in capital	295,372	2,492
Accumulated deficit	(181,840)	(115,446)
Total stockholders' equity (deficit)	113,535	(112,954)
Total liabilities, preferred stock and stockholders' equity (deficit)	<u>\$ 139,807</u>	<u>\$ 97,076</u>

The accompanying notes are an integral part of these consolidated financial statements.

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Lexeo Therapeutics, Inc.
Consolidated Statements of Operations and Comprehensive Loss
(In thousands, except share and per share amounts)

	Year Ended December 31,	
	2023	2022
Revenue		
Grant revenue	\$ —	\$ 654
Total revenue	<u>—</u>	<u>654</u>
Operating expenses		
Research and development	53,130	49,162
General and administrative	15,383	12,001
Total operating expenses	<u>68,513</u>	<u>61,163</u>
Operating loss	<u>(68,513)</u>	<u>(60,509)</u>
Other income and expense		
Loss on fair value adjustment to convertible SAFE Note	(530)	—
Other income (expense), net	(13)	(2)
Interest expense	(205)	(91)
Interest income	2,867	1,325
Total other income and expense	<u>2,119</u>	<u>1,232</u>
Loss from operations before income taxes	<u>(66,394)</u>	<u>(59,277)</u>
Income taxes	<u>—</u>	<u>—</u>
Net loss and comprehensive loss	<u>\$ (66,394)</u>	<u>\$ (59,277)</u>
Net loss per common share, basic and diluted	<u>\$ (12.40)</u>	<u>\$ (36.36)</u>
Weighted average number of shares outstanding used in computation of net loss per common share, basic and diluted	5,354,368	1,630,348

The accompanying notes are an integral part of these consolidated financial statements.

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Lexeo Therapeutics, Inc.
Consolidated Statements of Convertible Preferred Stock and Stockholders' Equity (Deficit)

(In thousands, except share amounts)

	Convertible Preferred Stock		Common Stock		Additional Paid-in Capital	Accumulated Deficit	Total Stockholders' Equity (Deficit)
	Shares	Amount	Shares	Amount			
Balances at December 31, 2021	143,653,545	\$ 185,013	1,580,651	\$ —	\$ 567	\$ (56,169)	\$ (55,602)
Proceeds from issuance of series A convertible preferred stock	—	20	—	—	—	—	—
Exercise of stock options	—	—	68,083	—	332	—	332
Proceeds received from early exercise of unvested stock options subject to repurchase and recorded as deposit liabilities, net of amounts reclassified from deposit liabilities upon the vesting of early-exercised stock options previously subject to repurchase	—	—	(41,549)	—	(188)	—	(188)
Stock-based compensation expense	—	—	—	—	1,781	—	1,781
Net loss	—	—	—	—	—	(59,277)	(59,277)
Balances at December 31, 2022	143,653,545	\$ 185,033	1,607,185	\$ —	\$ 2,492	\$ (115,446)	\$ (112,954)
Exercise of stock options	—	—	11,916	—	51	—	51
Amounts reclassified from deposit liabilities upon the vesting of early-exercised stock options previously subject to repurchase, net of proceeds received from early exercise of unvested stock options subject to repurchase and recorded as deposit liabilities	—	—	19,442	—	87	—	87
Conversion of SAFE Note into common stock in connection with initial public offering, net of issuance costs of \$113	—	—	411,815	—	4,417	—	4,417
Issuance of common stock upon initial public offering and subsequent partial exercise of underwriters' option to purchase additional shares, net of underwriting discounts, commissions and offering costs of \$11,285	—	—	10,139,656	1	100,250	—	100,251
Conversion of convertible preferred stock into common stock in connection with initial public offering, net of issuance costs of \$519	(143,653,545)	(185,033)	14,456,364	2	185,032	—	185,034
Stock-based compensation expense	—	—	—	—	3,043	—	3,043
Net loss	—	—	—	—	—	(66,394)	(66,394)
Balances at December 31, 2023	—	—	26,646,378	\$ 3	\$ 295,372	\$ (181,840)	\$ 113,535

The accompanying notes are an integral part of these consolidated financial statements.

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Lexeo Therapeutics, Inc. Consolidated Statements of Cash Flows (In thousands)

	Year Ended December 31,	
	2023	2022
Cash flows from operating activities:		
Net loss	\$ (66,394)	\$ (59,277)
Adjustments to reconcile net loss to net cash (used in) provided by operating activities:		
Reduction in the carrying amount of ROU assets, operating	1,266	877
Reduction in the carrying amount of ROU assets, finance	306	148
Stock based compensation expense	3,043	1,781
Depreciation and amortization expense	271	120
Change in fair value of convertible SAFE Note liability	530	—
Changes in operating assets and liabilities:		
Prepaid expenses and other current assets	990	(1,889)
Security deposits	—	2
Accounts payable	(180)	(426)
Change in deferred income	—	(46)
Accrued expenses and other current liabilities	2,440	4,476
Taxes payable	(349)	(22)
Lease liability, operating	(1,414)	(298)
Lease liabilities, finance	(5)	(6)
Net cash used in operating activities	<u>(59,496)</u>	<u>(54,560)</u>
Cash flows from investing activities:		
Purchase of internal use software	(50)	(229)
Purchase of property and equipment	(115)	(672)
Net cash used in investing activities	<u>(165)</u>	<u>(901)</u>
Cash flows from financing activities:		
Proceeds from issuance of series A convertible preferred stock	—	20
Proceeds from exercise of stock options	51	332
Payments on finance leases	(425)	(163)
Payments of issuance costs on convertible SAFE Note	(86)	—
Proceeds from issuance of convertible SAFE Note	4,000	—
Proceeds from issuance of common stock upon initial public offering, net of underwriters discounts, commissions and other offering costs	100,251	—
Net cash provided by financing activities	<u>103,791</u>	<u>189</u>
Net change in cash, cash equivalents and restricted cash	44,130	(55,272)
Total cash, cash equivalents and restricted cash at beginning of period	80,588	135,860
Total cash, cash equivalents and restricted cash at end of period	<u>\$ 124,718</u>	<u>\$ 80,588</u>
Cash and cash equivalents at beginning of period	77,335	135,860
Restricted cash included in long-term assets at beginning of period	3,253	—
Total cash, cash equivalents and restricted cash at beginning of period	<u>\$ 80,588</u>	<u>\$ 135,860</u>
Cash and cash equivalents at end of period	121,466	77,335
Restricted cash included in long-term assets at end of period	3,252	3,253
Total cash, cash equivalents and restricted cash at end of period	<u>\$ 124,718</u>	<u>\$ 80,588</u>
Supplemental disclosures of cash flow information		
Interest paid	\$ 176	\$ 77
Income taxes paid	—	—
Supplemental disclosure of non-cash activities		
Deferred financing costs included in accounts payable and accrued expenses	\$ 1,105	\$ —
Issuance costs related to convertible debt included in accounts payable and accrued expenses	\$ 50	\$ —
Purchase of property and equipment included in accounts payable and accrued expenses	\$ 141	\$ 43
Operating lease right-of-use assets and operating lease liabilities recognized	\$ —	\$ 11,585
Finance lease right-of use assets and finance lease liabilities recognized	\$ —	\$ 2,364
Conversion of convertible preferred stock into common stock in connection with initial public offering, net of issuance costs	\$ 185,032	\$ —
Conversion of SAFE Note into common stock in connection with initial public offering, net of issuance costs	\$ 4,417	\$ —
Amounts reclassified from deposit liabilities upon the vesting of early-exercised stock options previously subject to repurchase, (net of proceeds received from early exercise of unvested stock options subject to repurchase and recorded as deposit liabilities)	\$ 87	\$ (188)

The accompanying notes are an integral part of these consolidated financial statements.

Lexeo Therapeutics, Inc.

Notes to Consolidated Financial Statements

(Table amounts in thousands, except share and per share amounts)

1. Description of Business and Basis of Presentation

Description of Business—Lexeo Therapeutics, Inc. (the “Company”), together with its consolidated subsidiary, Stelios Therapeutics Inc. (“Stelios”), until such subsidiary was merged into the Company on December 15, 2022 and ceased to exist, is a clinical stage genetic medicine company with a focus on hereditary and acquired diseases of high unmet need. The Company’s investigational therapies have the potential to offer gene therapy-based treatments to address many diseases that have eluded today’s existing drug delivery platforms. The Company utilizes adeno-associated viruses (“AAV”) that have been engineered to transfer genes to patients. The Company’s therapeutic investigational treatments include gene therapies primarily in the early clinical and late pre-clinical stages of research and development.

The Company is located in New York, NY and was first formed on February 17, 2017, as an LLC under the laws of the State of Delaware under the legal name Lexeo Therapeutics, LLC. The Company filed and executed a certificate of conversion to corporation on November 20, 2020, to convert the LLC to Lexeo Therapeutics, Inc, a Delaware corporation. All of the Company’s tangible assets are held in the United States (“U.S.”).

Basis of Presentation and Principles of Consolidation—The accompanying consolidated financial statements reflect the operations of the Company and its wholly-owned subsidiary that have been prepared in conformity with generally accepted accounting principles in the United States of America (“GAAP”). Any reference in these notes to applicable guidance is meant to refer to the authoritative GAAP as found in the Accounting Standards Codification (“ASC”) and Accounting Standards Updates (“ASU”) of the Financial Accounting Standards Board (“FASB”). The accounts of the wholly owned subsidiary are included in these consolidated financial statements and accompanying notes, and the Company has no unconsolidated subsidiaries. All intercompany balances and transactions have been eliminated in consolidation.

A 10.594230-for-1 reverse share split of the Company’s series A convertible preferred stock, series B convertible preferred stock, common stock, and options to purchase common stock under the Company’s 2021 Equity Incentive Plan, as well as corresponding adjustments in the respective conversion prices of the series A convertible preferred stock and series B convertible preferred stock, was effected on October 13, 2023 as approved by the Company’s board of directors (the “Board of Directors”) and its shareholders (the “Stock Split”). The Stock Split reduced the number of shares of the Company’s authorized, issued and outstanding common stock, as well as the numbers of shares reserved and available for future issuance and underlying outstanding options to purchase common stock under its 2021 Equity Incentive Plan, on a 10.594230-for-1 basis. As such, all references to series A convertible preferred stock and series B convertible preferred stock conversion ratios, conversion share and per share amounts, and post-conversion share and per share amounts, as well as common stock option, option per common share, common share and common per share amounts, in these consolidated financial statements and accompanying notes have been retroactively restated to reflect the Stock Split and the Stock Split’s effect on the respective series A convertible preferred stock and series B convertible preferred stock conversion ratios for each series of convertible preferred stock. The Stock Split did not affect the par values per share.

Need for Additional Capital—Since inception, the Company has incurred net losses and negative cash flows from operations, including net losses of \$66.4 million and \$59.3 million during the years ended December 31, 2023 and December 31, 2022, respectively. As of December 31, 2023, the Company had cash and cash equivalents of \$121.5 million and an accumulated deficit of \$181.8 million and expects to incur substantial operating losses and negative cash flows from operations for the foreseeable future. During the years ended December 31, 2021 and December 31, 2020, the Company raised aggregate total net proceeds of \$185.0 million in connection with the issuance of series A and series B convertible preferred stock. During the year ended

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December 31, 2023 the Company raised \$100.3 million of total net proceeds in connection with the closing of its planned initial public offering (“IPO”) on November 7, 2023 and subsequent partial exercise of the underwriters’ option to purchase additional shares (see Note 8), as well \$3.9 million of net proceeds from the issuance of a convertible Simple Agreement for Future Equity (“SAFE”) note (the “SAFE Note”) in August 2023 (see Note 6). Management estimates that the Company’s current cash and cash equivalents balance is sufficient to fund its operations for at least 12 months from the issuance date of these consolidated financial statements.

If the Company is unable to obtain additional funding before achieving sufficient profitability and positive cash flows from operations, if ever, the Company will be forced to delay, reduce or eliminate some or all of its research and development programs, which could adversely affect its business prospects, or the Company may be unable to continue operations. Although management continues to pursue these plans, there is no assurance that the Company will be successful in obtaining sufficient funding on terms acceptable to the Company to fund continuing operations, if at all.

Risks and Uncertainties—The Company is subject to risks and uncertainties common to early-stage companies in the biopharmaceutical industry, including, but not limited to, successful discovery and development of its product candidates, development by competitors of new technological innovations, dependence on key personnel, the ability to attract and retain qualified employees, protection of proprietary technology, compliance with governmental regulations, the ability to secure additional capital to fund operations, and commercial success of its product candidates. Any of the Company’s current product candidates and future product candidates that it may develop will require extensive nonclinical and clinical testing and regulatory approval prior to commercialization. These efforts require significant amounts of additional capital, adequate personnel, infrastructure, and extensive compliance- reporting capabilities. Even if the Company’s product development efforts are successful, it is uncertain when, if ever, the Company will realize significant revenue from product sales.

2. Summary of Significant Accounting Policies

Use of Estimates—The preparation of the financial statements in accordance with U.S. GAAP requires management to make estimates and assumptions that affect the reported amounts of assets and liabilities and disclosure of contingent assets and liabilities at the date of the financial statements, and the reported amounts of income and expense during the reporting period. The most significant estimates relate to the accruals of research and development costs, including accruals of research contract costs, and assumptions used to estimate the fair value of the Company’s stock option awards and, prior to its IPO, to determine the fair value of its common stock. Management evaluates its estimates and assumptions on an ongoing basis using historical experience and other factors, including the current economic environment, and makes adjustments when facts and circumstances dictate. These estimates are based on information available as of the date of the financial statements; therefore, actual results could differ from those estimates.

Cash and Cash Equivalents and Restricted Cash—The Company considers all highly liquid investments with a remaining maturity when purchased of three months or less to be cash equivalents. Cash equivalents are reported at carrying values, which approximated their fair values and are based on quoted prices in active markets for identical securities. At December 31, 2023 and December 31, 2022, the Company’s cash equivalents were held in a money market fund. As of December 31, 2023 and 2022, cash consists of cash on deposit with U.S. banks denominated in U.S. dollars, and restricted cash consists of cash on deposit with U.S. banks denominated in U.S. dollars for which its use is restricted and is classified as non-current in the Company’s consolidated balance sheet according to the timing of maturity, and is associated with collateral for letters of credit issued in connection with its operating lease right-of-use and finance lease right-of-use assets and corresponding lease liabilities. Cash is stated at its historical carrying amount, which approximates fair value due to its short-term nature. Restricted cash is stated at its historical carrying amount, which approximates fair value. The Company regularly maintains cash and cash equivalents and restricted cash balances with financial institutions that exceed Federal Deposit Insurance Corporation insurance limits.

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Net Loss per Share—The Company follows the two-class method when computing net income (loss) per common share as the Company has issued shares that meet the definition of participating securities. The two-class method determines net income (loss) per common share for each class of common and participating securities according to dividends declared or accumulated and participation rights in undistributed earnings. The two-class method requires income (loss) available to common stockholders for the period to be allocated between common and participating securities based upon their respective rights to receive dividends as if all income for the period had been distributed. The Company considers its convertible preferred stock to be participating securities as, in the event a dividend is paid on common stock, the holders of these securities would be entitled to receive dividends on a basis consistent with the common stockholders. The Company also considers the shares issued upon the early exercise of stock options that are subject to repurchase to be participating securities because holders of such shares have non-forfeitable dividend rights in the event a dividend is paid on common stock. There is no allocation required under the two-class method during periods of loss since the participating securities do not have a contractual obligation to share in the losses of the Company.

Basic net income (loss) per common share is computed by dividing the net income (loss) per common share by the weighted-average number of common shares outstanding for the period. Diluted net income (loss) per common share is computed by adjusting net income (loss) to reallocate undistributed earnings based on the potential impact of dilutive securities. Diluted net loss per common share is computed by dividing the diluted net loss by the weighted-average number of common shares outstanding for the period, including potential dilutive common shares. For the purposes of this calculation, shares of convertible preferred stock are considered potential dilutive common shares.

In periods in which the Company reported a net loss, diluted net loss per common share was the same as basic net loss per common share since dilutive common shares were not assumed to have been issued if their effect was anti-dilutive. During (i) the year ended December 31, 2023, potential common shares related to the conversion of the convertible SAFE Note, which converted into 411,815 common shares in connection with the Company's IPO on November 2, 2023 (see Note 6), (ii) the years ended December 31, 2023 and December 31, 2022, 8,070,027 potential common shares related to the conversion of series A convertible preferred stock and 5,489,573 potential common shares related to the conversion of series B convertible preferred stock, which converted into 8,070,027 common shares and 6,386,337 common shares, respectively, in connection with the Company's IPO on November 7, 2023 (see Note 7), and (iii) the years ended December 31, 2023 and December 31, 2022, 2,415,740 and 1,916,194 potential common shares, respectively, related to the exercise of outstanding stock options (see Note 9), were excluded from the computation of diluted net loss per common share because including them would have had an anti-dilutive effect as the Company reported net losses for those periods.

Deferred Offering Costs—The Company capitalizes certain legal, accounting and other third-party fees that are directly associated with equity financings as deferred offering costs until such financings are consummated. After consummation of the equity financing, these costs are recorded as a reduction of the proceeds from the offering, either as a reduction of the carrying value of preferred stock or in stockholders' equity (deficit) as a reduction of additional capital generated as a result of the offering. Should the equity financing be abandoned, the deferred offering costs would be expensed immediately as a charge to operating expenses in the consolidated statement of operations and comprehensive loss. The Company had no deferred offering costs recorded as of December 31, 2023 or December 31, 2022.

Accounts Receivable and Allowance for Doubtful Accounts—Accounts receivable are recorded net of allowances for doubtful accounts. The Company does not have any accounts receivable but in certain cases had a grant receivable balance. For the Company to be in a receivable position related to grants, the cumulative total spend on grants must exceed the cumulative cash receipts if there remains additional award money to be collected.

Classification of Convertible Preferred Stock—Until the closing of the Company's IPO on November 7, 2023, the holders of the series A and series B convertible preferred stock had certain liquidation rights in the event of a

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deemed liquidation that, in certain situations, were not solely within the control of the Company and could have called for the redemption of the then outstanding series A convertible preferred stock. Therefore, the series A and series B convertible preferred stock were classified outside of the stockholders' equity (deficit) on the Company's consolidated balance sheets. The carrying value of the convertible preferred stock was not subsequently remeasured to the redemption value until the contingent redemption events were considered to be probable of occurring.

Grant Revenue—Grants received are recognized as grant revenue in the statements of operations as and when they are earned for the specific research and development projects for which these grants are designated. Grant payments received in excess of grant revenue earned are recognized as deferred grant revenue on the Company's consolidated balance sheet and grant revenue earned in excess of grant payments received is recognized as grant receivable on the Company's consolidated balance sheet.

Research and Development—Research and development costs are expensed as incurred. Research and development expenses consist of costs incurred to discover, research, and develop drug candidates, including personnel expenses, allocated facility-related and depreciation expenses, and third-party license fees. Costs incurred to obtain technology licenses are charged immediately to research and development expense if the technology licensed has not reached technological feasibility and has no alternative future use.

General and Administrative—General and administrative expenses consist primarily of the cost of employees to engage in corporate functions, such as finance and accounting, information technology, human resources, and legal and executive management. General and administrative expenses also include rent occupancy costs, office expenses, depreciation and amortization, other general overhead costs, insurance premiums, professional service fees, and costs related to regulatory and litigation matters.

Stock Based Compensation Expense—The Company accounts for stock-based payment awards granted to employees and non-employees as stock-based compensation expense at fair value. The Company's stock-based payments include stock options and grants of common stock, including common stock subject to vesting. The measurement date for employee awards is the date of grant, and stock-based compensation costs are recognized as expense over the employees' requisite service period, which is the vesting period, on a straight-line basis. The measurement date for non-employee awards is the date of grant without changes in the fair value of the award. Stock-based compensation costs for non-employees are recognized as expense over the vesting period on a straight-line basis. Stock-based compensation expense is classified in the accompanying statement of operations based on the function to which the related services are provided. As the Company is permitted to repurchase shares legally issued for unvested stock options exercised at their exercise price, (i) cash received for unvested stock options exercised is recorded as a deposit liability as a reclassification from additional paid-in capital in the Company's consolidated balance sheet, which is relieved to additional paid-in capital as such awards vest, (ii) stock based compensation expense is recognized over the requisite service period for each such award, and (iii) the amount and number of shares of common stock outstanding in the Company's consolidated balance sheet and consolidated statement of convertible preferred stock and stockholders' equity (deficit) are reduced until such awards vest. The Company recognizes stock-based compensation expense for the portion of awards that have vested. Forfeitures are recorded as they occur. The Company recognizes the cumulative effect of changes in the probability outcomes in the period in which the changes occur.

The fair value of each stock option grant is estimated on the date of grant using the Black-Scholes option-pricing model. The Company has historically been a private company and lacks company-specific historical and implied volatility information for stock options granted prior to the Company's IPO. Therefore, the Company estimated its expected stock volatility entirely based on the historical volatility of a publicly traded set of peer companies for stock options granted prior to its IPO, and estimates its expected stock price volatility primarily based on the historical volatility of a publicly traded set of peer companies for stock options granted since its IPO, each for a period equal to the expected life of the stock options granted, and expects to continue to do so until such time as it has adequate historical data regarding the volatility of its own traded stock price. The expected term of the

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Company's stock options has been determined utilizing the "simplified" method for awards that qualify as "plain-vanilla" options. The risk-free interest rate is determined by reference to the U.S. Treasury yield curve in effect at the time of grant of the award for time periods approximately equal to the expected term of the award. The expected dividend yield is zero as the Company has never paid cash dividends on its common stock and does not expect to pay any cash dividends in the foreseeable future.

Income Taxes—Prior to November 20, 2020, the Company was an LLC entity and elected to be treated under the Partnership provisions of the Internal Revenue Code. Accordingly, the LLC entity was not viewed as a tax-paying entity in any jurisdiction and all income and deductions of the LLC entity flowed through to the individual members and therefore no income taxes were recorded by the Company. On November 20, 2020, the Company converted to a C Corporation.

The Company accounts for income taxes under the asset and liability method pursuant to ASC 740, *Income Taxes*. Under this method, the Company recognizes deferred tax assets and liabilities for the future tax consequences attributable to differences between the financial statement carrying amounts of existing assets and liabilities and their respective tax bases and operating loss and tax credit carryforwards. Deferred tax assets and liabilities are measured using enacted tax rates expected to apply to taxable income in the years in which those temporary differences are expected to be recovered or settled. The effect on deferred tax assets and liabilities of a change in tax rates is recognized in income in the period that includes the enactment date. A valuation allowance is recorded for deferred tax assets if it is more likely than not that some portion or all of the deferred tax assets will not be realized based on all available positive and negative evidence. Under the Tax Cuts and Jobs Act of 2017, the Company is required to capitalize, and subsequently amortize, research and development expenses over five years for research activities conducted in the U.S. and over fifteen years for research activities conducted outside of the U.S commencing in 2022. The capitalization of research and development expenses during the year resulted in a decrease to our operating loss generation during the years ended December 31, 2023 and December 31, 2022 as compared to prior periods (see Note 11). As of December 31, 2023 and December 31, 2022, the Company continues to maintain a full valuation allowance against its deferred tax assets.

The Company recognizes a tax benefit only if it is more likely than not the tax position will be sustained on examination by the local taxing authorities, based on the technical merits of the position. The tax benefits recognized in the financial statements from such positions are then measured based on the largest benefit greater than 50% likelihood of being realized upon settlement with the related tax authority. The changes in recognition or measurement are reflected in the period in which the change in judgment occurs. As of December 31, 2023 and December 31, 2022, the Company has not identified any uncertain tax positions.

The Company records interest and penalties related to uncertain tax positions in the provision for income taxes.

Fair Value Measurements—Fair value is defined as the price that would be received to sell an asset or paid to transfer a liability in an orderly transaction between market participants at the measurement date. The three levels of inputs that may be used to measure fair value are as follows:

Level 1—Inputs are quoted prices (unadjusted) in active markets for identical assets or liabilities that the Company has the ability to access at the measurement date.

Level 2—Inputs are observable, unadjusted quoted prices in active markets for similar assets or liabilities, unadjusted quoted prices for identical or similar assets or liabilities in markets that are not active, or other inputs that are observable or can be corroborated by observable market data for substantially the full term of the related assets or liabilities.

Level 3—Inputs are unobservable inputs for the asset or liability.

Concentrations of Credit Risk—Financial instruments that potentially subject the Company to concentrations of credit risk consist primarily of cash and cash equivalents and restricted cash. The Company's cash and

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restricted cash balances exceed Federal Deposit Insurance Corporation insurance limits, and the Company's cash equivalents consist of investments in a U.S. government money market fund. The Company's cash and cash equivalents and restricted cash is held with large financial institutions that management believes to be of high credit quality. To date, the Company has not recognized any losses caused by uninsured balances.

Segment Information—The Company manages its operations as a single segment for the purposes of assessing performance and allocating resources. The Company is focused on preclinical and clinical stage gene therapies, and specifically on hereditary and acquired diseases of high unmet need. The Company's chief operating decision maker ("CODM") reviews the Company's financial information on an aggregated basis for purposes of assessing performance and allocating resources. All assets are held in the United States. The Company has not earned any product revenue through December 31, 2023.

Property and Equipment—Property and equipment are stated at cost less accumulated depreciation and amortization. Depreciation and amortization is recognized using the straight-line method over the estimated useful life of each asset. Such costs are periodically reviewed for recoverability when impairment indicators are present. Such indicators include, among other factors, unused capacity, market value declines, and technology obsolescence. Recorded values of asset groups of equipment that are not expected to be recovered through undiscounted future net cash flows are written down to current fair value, which generally is determined from estimated discounted future net cash flows (assets held for use) or net realizable value (assets held for sale).

Costs for capital assets not yet placed into service are capitalized as construction-in-progress and depreciated once placed into service. Upon retirement or sale, the cost of assets disposed of and the related accumulated depreciation and amortization are removed from the accounts and any resulting gain or loss is included in loss from operations. Expenditures for repairs and maintenance that do not improve or extend the life of the respective assets are charged to expense in the period incurred. See Note 5 for information regarding the Company's capitalized operating and finance lease right-of-use assets.

The following is the summary of property and equipment and related accumulated depreciation as of December 31, 2023, and December 31, 2022 (years not stated in thousands):

	Useful Life	December 31, 2023	December 31, 2022
Internal use software	3 years	\$ 296	\$ 245
Furniture and fixtures	5 years	380	375
Lab equipment	7 years	514	279
Leasehold improvements	7 years	247	232
Total property and equipment		1,437	1,131
Less: accumulated depreciation and amortization		(381)	(133)
Total property and equipment, net		<u>\$ 1,056</u>	<u>\$ 998</u>

Leases—In accordance with ASC 842, *Leases*, the Company determines if an arrangement is or contains a lease at inception. A contract is or contains a lease if the contract conveys the right to control the use of an identified asset for a period of time in exchange for consideration. The Company classifies leases at the lease commencement date as operating or finance leases and records a right-of-use asset and a lease liability on the consolidated balance sheet for all leases with an initial lease term of greater than 12 months. Leases with an initial term of 12 months or less are not recorded in the balance sheet, but payments are recognized as expense on a straight-line basis over the lease term. The Company has elected not to recognize leases with terms of 12 months or less.

A lease qualifies as a finance lease if any of the following criteria are met at the inception of the lease: (i) there is a transfer of ownership of the leased asset to the Company by the end of the lease term, (ii) the Company holds

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an option to purchase the leased asset that it is reasonably certain to exercise, (iii) the lease term is for a major part of the remaining economic life of the leased asset, (iv) the present value of the sum of lease payments equals or exceeds substantially all of the fair value of the leased asset, or (v) the nature of the leased asset is specialized to the point that it is expected to provide the lessor no alternative use at the end of the lease term. All other leases are recorded as operating leases.

The Company enters into contracts that contain both lease and non-lease components. Non-lease components may include maintenance, utilities, and other operating costs. The Company combines the lease and non-lease components of fixed costs in its lease arrangements as a single lease component. Variable costs, such as utilities or maintenance costs, are not included in the measurement of right-of-use assets and lease liabilities, but rather are expensed when the event determining the amount of variable consideration to be paid occurs.

Operating lease right-of-use assets and lease liabilities are recognized at the lease commencement date based on the present value of the lease payments over the lease term using the discount rate implicit in the lease. If the implicit rate is not readily determinable, the Company utilizes an estimate of its incremental borrowing rate based upon the available information at the operating lease commencement date. Operating lease right-of-use assets are further adjusted for prepaid or accrued lease payments. Operating lease payments are expensed using the straight-line method as an operating expense over the lease term. The Company's lease terms may include options to extend or terminate the lease when it is reasonably certain that the Company will exercise that option. Finance lease right-of-use assets and lease liabilities are recognized upon receipt of the assets based on their fair values, and are amortized to depreciation expense using the straight-line method over the estimated useful lives of the related assets. Finance lease liability payments are bifurcated into (i) a portion that is recorded as interest expense and (ii) a portion that reduces the finance liability associated with the lease, according to the interest rates implicit in the leases.

Certain of the Company's leases include options to extend or terminate the lease. The amounts determined for the Company's right-of-use assets and lease liabilities generally do not assume that renewal options or early-termination provisions, if any, are exercised, unless it is reasonably certain that the Company will exercise such options.

Recent Accounting Pronouncements Not Yet Adopted—In December 2023, the FASB issued ASU No. 2023-09, *Income Taxes* (Topic 740). ASU No. 2023-09 requires disaggregation of the effective tax rate reconciliation into standard categories, enhances disclosure of income taxes paid, and modifies other income tax-related disclosures. ASU No. 2023-09 will be effective for the Company starting in annual periods in 2025, with early adoption permitted. The Company is currently assessing the impact of adopting this guidance on its consolidated financial statements.

In November 2023, the FASB issued ASU No. 2023-07, *Segment Reporting—Improving Reportable Segment Disclosures* (Topic 280). ASU No. 2023-07 requires disclosures to include significant segment expenses that are regularly provided to the CODM, a description of other segment items by reportable segment, and any additional measures of a segment's profit or loss used by the CODM when deciding how to allocate resources. ASU No. 2023-07 also requires all annual disclosures currently required by Topic 280 to be included in interim periods. ASU No. 2023-07 is effective for the Company starting in annual periods in 2024 and interim periods in 2025, with early adoption permitted and retrospective application required to all prior periods presented in the financial statements. The Company is currently assessing the impact of adopting this guidance on its consolidated financial statements but does not expect it to have a material impact.

3. Fair Value Measurements

The Company's cash equivalents consist of investments in a U.S. government money market fund stated at carrying value, which approximates fair value and is based on quoted prices in active markets for identical securities. Cash is stated at carrying value, which approximates fair value due to its short-term nature. The

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carrying values of the Company's prepaid expenses, other current assets, accounts payable and accrued expenses approximate their fair values due to the short-term nature of these assets and liabilities. The carrying value of the Company's convertible SAFE Note, gross of unamortized discount, was estimated using a probability-weighted present value model, which involved the use of significant unobservable inputs (see Note 6). There have been no transfers between fair value levels during the years ended December 31, 2023 and December 31, 2022.

The following table presents information about the Company's financial assets and liabilities measured at fair value on a recurring basis and indicates the level of the fair value hierarchy utilized to determine such fair values:

	As of December 31, 2023:			Total
	Level 1	Level 2	Level 3	
Assets:				
Cash equivalents (money market)	\$ 102,484	\$ —	\$ —	\$ 102,484
	<u>\$ 102,484</u>	<u>\$ —</u>	<u>\$ —</u>	<u>\$ 102,484</u>
	As of December 31, 2022:			Total
	Level 1	Level 2	Level 3	
Assets:				
Cash equivalents (money market)	\$ 74,802	\$ —	\$ —	\$ 74,802
	<u>\$ 74,802</u>	<u>\$ —</u>	<u>\$ —</u>	<u>\$ 74,802</u>

The following table sets forth a roll forward of changes in the fair value of financial liabilities, gross of unamortized discount, classified as Level 3 in the fair value hierarchy:

	Convertible SAFE Note, gross
Beginning balance at December 31, 2022	\$ —
Convertible SAFE Note issuance at August 24, 2023	4,000
Change in estimated fair value since issuance	530
Conversion of SAFE Note into common stock in connection with initial public offering	(4,530)
Ending balance at December 31, 2023	<u>\$ —</u>

The valuation models used to estimate the fair value of the Company's convertible SAFE Note considered the estimated probabilities of each of qualified equity financing, public offering, and change of control events, together with corresponding estimated periods of time from each valuation date until the achievements of each such event. Future values were converted to present value using a discount rate appropriate for probability-adjusted cash flows. The ranges of the significant unobservable inputs used in the valuation model to estimate the fair value of the Company's convertible SAFE Note that was categorized within Level 3 of the fair value hierarchy as of August 24, 2023 (issuance) until November 2, 2023 (settlement) were as follows (not stated in thousands):

Probability of meeting qualified equity financing event	10.0% - 20.0%
Probability of meeting public offering event	75.0% - 85.0%
Probability of meeting change in control event	5%
Time until qualified equity financing event (in years)	0.17 - 0.27
Time until public offering event (in years)	0.08 - 0.19
Time until change in control event (in years)	0.50 - 0.60

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In connection with the closing of the Company's IPO on November 7, 2023, the underwriters of the IPO were granted an option for a period of 30 days to purchase up to 1,363,636 additional shares from the Company at the public offering price of \$11.00 per share less the underwriting discount, of which 1,048,746 shares were exercised (see Note 8). The total grant date fair value of this option of approximately \$1.2 million, or \$0.85 per share, was recorded as an offset to additional paid-in capital and was estimated using a Black-Scholes model incorporating the following assumptions (not stated in thousands):

Stock price	\$ 11.00
Exercise price	\$ 11.00
Weighted average risk-free interest rate	5.53%
Expected term (in years)	0.08
Expected volatility	65.88%
Expected dividend yield	0.00%

4. Accrued Expenses and Other Current Liabilities

Accrued expenses and other current liabilities consisted of the following:

	December 31, 2023	December 31, 2022
Accrued research and development expenses	\$ 6,384	\$ 4,755
Accrued payroll expenses	242	796
Accrued professional fees	1,078	629
Other current liabilities	169	240
Total accrued expenses and other current liabilities	<u>\$ 7,873</u>	<u>\$ 6,420</u>

5. Leases

Operating Lease Right-of-Use Asset

In January 2022, the Company entered into a lease agreement for an office facility and laboratory space in New York, New York that commenced in April 2022 and ends in July 2029 with an additional five-year option to extend the lease beyond July 2029 at the then-prevailing effective market rental rate. Upon commencement of this lease, the Company recorded operating lease right-of-use assets and operating lease liabilities of \$11.6 million based on the present value of payments over the lease term using an estimated incremental borrowing rate of 8.53% in accordance with the provisions of ASC 842. In connection with the Company's lease of office space and laboratory space, the Company provided a security deposit to the landlord in the form of a letter of credit totaling \$1.2 million. The cash collateralizing the letter of credit was included in long-term restricted cash in the Company's consolidated balance sheets as of December 31, 2023, and December 31, 2022. This lease was classified as an operating lease in accordance with the provisions of ASC 842. The Company did not recognize any right-of-use assets and lease liabilities associated with the potential option to renew or extend. The Company's operating lease agreement does not contain any significant residual value guarantees or restrictive covenants.

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The remaining lease terms and payment terms as of December 31, 2023 and December 31, 2022 were 5.6 years and 6.6 years, respectively. The components of this operating lease were as follows:

	Year Ended December 31,	
	2023	2022
Operating lease expense	\$2,147	\$1,610
Variable lease expense	355	185
Total operating lease expense	\$ 2,502	\$ 1,795
Cash paid for amounts included in the measurement of lease liabilities, included in operating cash flows	\$2,294	\$1,032

The following table provides a reconciliation of the Company's remaining undiscounted contractual rent obligations due within each year ended December 31 to the operating lease liabilities recognized as of December 31, 2023:

Year ended December 31	Operating Leases
2024	\$ 2,112
2025	2,152
2026	2,206
2027	2,261
2028	2,318
Thereafter	1,372
Total lease payments	12,421
Less: present value adjustment	(2,548)
Total operating lease liabilities	<u>\$ 9,873</u>
Included in the consolidated balance sheet:	
Current portion of lease liabilities—operating	2,087
Non-current portion of lease liabilities—operating	7,786
Total operating lease liabilities	<u>\$ 9,873</u>

Equipment Finance Leases

Commencing in April 2022, the Company leases certain laboratory equipment under financing arrangements accounted for as finance leases in accordance with the provisions of ASC 842 that are classified in the Company's consolidated balance sheet as finance lease liabilities with related right-of-use assets recorded and depreciated on a straight-line basis over the estimated useful life of 7 years. In connection with the Company's leases of laboratory equipment, the Company provided a security deposit to the lessor in the form of a letter of credit totaling \$1.9 million and assigned all rights and interests in the equipment to the lessor. The cash collateralizing the letter of credit is included in long-term restricted cash in the Company's consolidated balance sheet as of December 31, 2023. The total gross, accumulated amortization, and net book values of equipment finance lease right-of-use assets capitalized under such finance lease arrangements at December 31, 2023 were \$2.2 million, \$0.5 million and \$1.7 million, respectively. Under the terms of the equipment finance lease agreements executed through the issuance of these consolidated financial statements, the principal balances plus interest for the equipment are to be repaid in full after 60 monthly installments following lease commencement, with lease commencement dates ranging from April 1, 2022 to April 1, 2023, annual imputed interest rates ranging from 7.90% to 9.30%, and monthly installment payment amounts ranging from \$4,000 to \$18,000. The total aggregate monthly installment payment amount was \$49,000 for equipment finance lease agreements executed through the issuance date of these consolidated financial statements.

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The weighted-average remaining lease payment term, weighted-average remaining amortization term, and weighted-average effective interest rate for the Company's equipment finance lease agreements as of December 30, 2023 were 3.7 years, 5.7 years, and 8.59%, respectively. The weighted-average remaining lease payment term, weighted-average remaining amortization term, and weighted-average effective interest rate for the Company's equipment finance lease agreements as of December 31, 2022 were 4.6 years, 6.6 years, and 8.66%, respectively. The components of the equipment finance leases were as follows:

	Year Ended December 31,	
	2023	2022
Reduction in the carrying amount of ROU assets, finance	\$306	\$148
Interest on finance lease liabilities	176	77
Total finance lease expense	\$ 482	\$ 225
Cash paid for amounts included in the measurement of lease liabilities, included in financing cash flows	\$425	\$163

The following table provides a reconciliation of the Company's remaining equipment finance lease obligations due within each year ending December 31 to the equipment finance lease liabilities recognized at December 31, 2023:

Year ended December 31	Equipment Finance Leases
2024	\$ 531
2025	579
2026	579
2027	357
2028	11
Thereafter	—
Total lease payments	2,057
Less: imputed interest	(292)
Total finance lease liabilities	\$ 1,765
Included in the consolidated balance sheet:	
Current portion of lease liabilities—finance	518
Non-current portion of lease liabilities—finance	1,247
Total finance lease liabilities	\$ 1,765

6. Convertible SAFE Note

On August 24, 2023, the Company entered into a convertible SAFE Note with Sarepta Therapeutics, Inc. (the "Investor") in a principal amount of \$4.0 million bearing simple interest at an annual rate of 10% to be repaid (the "Cash-Out Amount") after one year in the absence of the occurrence of certain settlement events, including upon a qualified preferred stock equity financing, a public offering or a Special Purpose Acquisition Company ("SPAC") transaction, a change of control, or dissolution. The convertible SAFE Note was accounted for as a liability in accordance with the provisions of ASC Topic 480, *Distinguishing Liabilities from Equity*, which was initially and subsequently measured at fair value with changes in fair value recognized in earnings (see Note 3). Upon a qualified equity financing event, the convertible SAFE Note would have automatically converted into a number of shares of corresponding preferred stock based on a certain discount to the price of such preferred stock issued to the other investors applied to the then-current Cash-Out Amount. Upon the effective date of an IPO or a direct listing, or immediately prior to the closing of a SPAC transaction, the convertible SAFE Note would have

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automatically converted, and did automatically convert upon the effectiveness of the Company's IPO registration statement on November 2, 2023, into a number of shares of common stock based on a certain discount to the public offering price of such common stock applied to the then-current Cash-Out Amount (see Note 8). Upon a change of control, the Investor would have been entitled to receive the greater of (i) an amount equal to a certain premium applied to the then-current Cash-Out Amount, or (ii) the amount that would have been payable on the number of shares of the senior-most series of the Company's preferred stock after applying a certain discount to the original issue price of such senior-most preferred stock together with a certain premium applied to the then-current Cash-Out Amount (the greater of (i) and (ii), the "Conversion Amount"). Upon the one-year maturity of the convertible SAFE Note, or upon dissolution of the Company, the Investor was to be paid an amount equal to the Cash-Out Amount. Upon the event of a change of control or dissolution, the Investor's right to receive its Cash-Out Amount or its Conversion Amount would have been junior to payment of outstanding indebtedness and creditor claims, on par with payments for the Company's most senior series of preferred stock or other SAFE note holders, if any, and senior to payments for the Company's common stock and any other series or class of stock. The convertible SAFE Note did not maintain any voting rights.

In connection with the convertible SAFE Note, the Company incurred issuance costs of \$0.1 million that were recorded as a discount to the liability balance and amortized to interest expense over the term of the convertible SAFE Note. As of November 2, 2023, the estimated fair value of the convertible SAFE Note, gross of unamortized discount, increased by \$0.5 million to \$4.5 million (see Note 3), and the corresponding carrying value was \$4.4 million, net of unamortized discount, which automatically converted into 411,815 shares of the Company's common stock upon the declaration of effectiveness of the Company's IPO registration statement on November 2, 2023 (see Note 8).

7. Convertible Preferred Stock

As of December 31, 2022 and until the closing of the Company's IPO on November 7, 2023, the Company's certificate of incorporation authorized the Company to issue 143,653,546 shares of series A and series B convertible preferred stock each with a par value of \$0.0001 per share. Upon the closing of the Company's IPO on November 7, 2023, all 85,495,722 outstanding shares of the Company's series A convertible preferred stock and all 58,157,823 outstanding shares of the Company's series B convertible preferred stock converted into 8,070,027 shares and 6,386,337 shares of common stock, respectively, including 896,764 shares of common stock issued as a result of series B convertible preferred stock antidilution provisions (see Note 8).

As of December 31, 2022 and until the closing of the Company's IPO on November 7, 2023, convertible preferred stock consisted of the following:

	Convertible Preferred Stock Authorized	Convertible Preferred Stock Issued and Outstanding	Proceeds Received	Liquidation Preference	Common Stock Issuable Upon Conversion
Series A convertible preferred stock	85,495,722	85,495,722	\$ 85,496	\$ 85,496	8,070,027
Series B convertible preferred stock	58,157,824	58,157,823	100,060	100,060	5,489,573
	<u>143,653,546</u>	<u>143,653,545</u>	<u>\$ 185,556</u>	<u>\$ 185,556</u>	<u>13,559,600</u>

As of December 31, 2022 and until the closing of the Company's IPO on November 7, 2023, the rights and privileges of the holders of the convertible preferred stock were as follows:

Voting—Holders of preferred stock were to vote together with the holders of common stock as a single class and on an as converted to common stock basis.

Dividends—The holders of shares of series B convertible preferred stock were entitled to receive, as declared by the Company's Board of Directors, dividends per share at the rate of 8% of the issue price per share of series B

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convertible preferred stock, prior and in preference to any declaration or payment of any other dividend (other than dividends on shares of common stock payable in shares of common stock). After payment of dividends to the holders of shares of series B convertible preferred stock, the holders of shares of series A convertible preferred stock were entitled to receive, as declared by the Company's Board of Directors, dividends per share at the rate of 8% of the issue price per share of series A convertible preferred stock, prior and in preference to any declaration or payment of any other dividend (other than dividends on shares of common stock payable in shares of common stock).

Deemed Liquidation Event—Each of the following events was to be considered a “Deemed Liquidation Event” unless the holders of a majority of the outstanding shares of preferred stock and the holders of at least 64% of the outstanding shares of series B convertible preferred stock elected otherwise by written notice to be sent to the Company at least five business days prior to the effective date of any such event; (a) a merger or consolidation in which the Company is a constituent party or a subsidiary of the Company was a constituent party and the Company issued shares of its capital stock pursuant to such merger or consolidation, except: any such merger or consolidation involving the Company or a subsidiary in which the shares of capital stock of the Company outstanding immediately prior to such merger or consolidation continued to represent, or were converted into or exchanged for shares of capital stock that represented, immediately following such merger or consolidation, at least a majority by voting power of the capital stock of (1) the surviving or resulting company; or (2) if the surviving or resulting company was a wholly owned subsidiary of another company immediately following such merger or consolidation, the parent company of such surviving or resulting corporation; (b) (1) the sale, lease, transfer, exclusive license or other disposition, in a single transaction or series of related transactions, by the Company or any subsidiary of the Company of all or substantially all the assets of the Company and its subsidiaries taken as a whole, or (2) the sale or disposition (whether by merger, consolidation or otherwise, and whether in a single transaction or a series of related transactions) of one or more subsidiaries of the Company if substantially all of the assets of the Company and its subsidiaries taken as a whole were held by such subsidiary or subsidiaries, except where such sale, lease, transfer, exclusive license or other disposition was to a wholly owned subsidiary of the Company; or (c) the Corporation's completion of (1) a reverse merger into a public shell, or (2) a merger or consolidation with a special purpose acquisition company or its subsidiary in which the common stock (or similar securities) of the surviving or parent entity were publicly traded in a public offering pursuant to an effective registration statement under the 1933 Securities Act, as amended.

Liquidation Preference—In the event of any voluntary or involuntary liquidation, dissolution, or winding up of the Company, the holders of shares of series B convertible preferred stock were entitled to be paid out of the assets of the Company or, in the case of a Deemed Liquidation Event, out of the consideration payable to stockholders or the available proceeds, before any payment would be made to the holders of common stock or series A convertible preferred stock, an amount per share equal to the series B convertible preferred stock issue price of \$18.227267 per share (subject to adjustment in the event of any stock dividend, stock split, combination or other similar recapitalization), plus any dividends declared but unpaid. If upon any such liquidation, dissolution or winding up of the Company or Deemed Liquidation Event, the assets of the Company available for distribution to its stockholders was insufficient to pay the holders of shares of series B convertible preferred stock the full amount to which they were entitled, the holders of shares of series B convertible preferred stock would have shared ratably in any distribution of the assets available for distribution in proportion to the respective amounts which would otherwise be payable in respect of the shares held by them upon such distribution if all amounts payable on or with respect to such shares were paid in full (the “Series B Liquidation Preference”).

In the event of any voluntary or involuntary liquidation, dissolution, or winding up of the Company, after payment of the Series B Liquidation Preference, the holders of shares of series A convertible preferred stock were entitled to be paid out of the assets of the Company or, in the case of a Deemed Liquidation Event, out of the consideration payable to stockholders or the available proceeds, before any payment shall be made to the holders of common stock or series A convertible preferred stock, an amount per share equal to the series A convertible preferred stock issue price of \$10.594230 per share (subject to adjustment in the event of any stock

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dividend, stock split, combination or other similar recapitalization), plus any dividends declared but unpaid. If upon any such liquidation, dissolution or winding up of the Company or Deemed Liquidation Event, the assets of the Company available for distribution to its stockholders was insufficient to pay the holders of shares of series A convertible preferred stock the full amount to which they were entitled, the holders of shares of series A convertible preferred stock would have shared ratably in any distribution of the assets available for distribution in proportion to the respective amounts which would otherwise be payable in respect of the shares held by them upon such distribution if all amounts payable on or with respect to such shares were paid in full (the “Series A Liquidation Preference”).

In the event that there were additional assets to be distributed after payment of the Series A Liquidation Preference and the Series B Liquidation Preference, the holders of the series A convertible preferred stock and series B convertible preferred stock would have shared in the distribution along with common stockholders as if the shares of the series A convertible preferred stock and series B convertible preferred stock had converted to common stock immediately prior to the distribution, up to an amount, (a) taken together with the Series A Liquidation Preference, not to exceed \$31.782691 per share with respect to the series A convertible preferred stockholders; and (b) taken together with the Series B Liquidation Preference, not to exceed \$54.681802 per share with respect to the series B convertible preferred stockholders.

Redemption—The preferred stock did not contain a mandatory redemption provision. Upon a deemed liquidation event, the holders would have been paid their preference amounts according to their priority. Remeasurement to redemption value would only have been required if a deemed liquidation event was probable.

Conversion—Upon either (a) the closing of the sale of shares of common stock to the public at a price of at least \$22.784057 per share (subject to appropriate adjustment in the event of any stock dividend, stock split, combination or other similar recapitalization with respect to the common stock), in a firm-commitment underwritten public offering pursuant to an effective registration statement under the Securities Act of 1933, as amended, resulting in at least \$75,000,000 of gross proceeds to the Company and in connection with the offering of the common stock listed for trading on the Nasdaq Stock Market’s National Market, the New York Stock Exchange or another exchange or marketplace approved by the Company’s Board of Directors, including the approval of at least two (2) preferred directors (a “Qualified IPO”), which occurred on November 7, 2023, or (b) the date and time, or the occurrence of an event specified by vote or written consent of the Noteholders, then (i) all outstanding shares of convertible preferred stock were to automatically be converted into shares of common stock, at the then effective conversion rate.

8. Capital Stock

As of December 31, 2023, the Company’s amended and restated certificate of incorporation provided that the authorized capital stock of the Company was 510,000,000 shares consisting of 500,000,000 shares of common stock and 10,000,000 shares of undesignated preferred stock, both with a par value of \$0.0001 per share. As of December 31, 2023, 26,668,485 shares of the Company’s common stock authorized were issued, including 22,107 shares that were legally issued upon the early exercise of unvested stock options and that are excluded from the number of shares outstanding until the right to repurchase subsequently lapses upon vesting. Until the closing of the Company’s IPO on November 7, 2023, the voting, dividend and liquidation rights of the holders of the Company’s common shares were subject to and qualified by the rights, powers and preferences of the holders of the convertible preferred stock set forth in Note 7. Each common share entitles the holder to one vote on all matters submitted to a vote of the Company’s stockholders. Common stockholders are entitled to receive dividends, if any, as may be declared by the Company’s Board of Directors, and were subject to the preferential dividend rights of the convertible preferred stock until the closing of the Company’s IPO. No cash dividends have been declared or paid by the Company.

Upon the declaration of effectiveness of the Company’s IPO registration statement on November 2, 2023, the Company’s convertible SAFE Note automatically converted into 411,815 shares of common stock (see Note 6). Upon the closing of the Company’s IPO on November 7, 2023, the Company issued and sold 9,090,910 shares of

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its common stock, and subsequently, the underwriters partially exercised their associated 30-day option to purchase additional shares of common stock with 1,048,746 additional shares issued (see Note 3). The net proceeds to the Company from the IPO and subsequent partial exercise of the underwriters' 30-day option to purchase additional shares were approximately \$100.3 million based on the initial offering price of \$11.00 per share, after deducting underwriting discounts, commissions and offering expenses totaling \$11.3 million. Also upon the closing of the Company's IPO on November 7, 2023, all 85,495,722 outstanding shares of the Company's series A convertible preferred stock and all 58,157,823 outstanding shares of the Company's series B convertible preferred stock converted into 8,070,027 shares and 6,386,337 shares of common stock, respectively, including 896,764 shares of common stock issued as a result of series B convertible preferred stock antidilution provisions (see Note 7).

The Company had reserved the following number of shares of common stock for the exercise of outstanding stock options, future issuance of stock-based awards, and conversion of outstanding shares of convertible preferred stock:

	December 31, 2023	December 31, 2022
Shares of series A convertible preferred stock	—	8,070,027
Shares of series B convertible preferred stock	—	5,489,573
Options to purchase shares of common stock under the 2021 Plan and 2023 Plan	2,415,740	1,916,194
Shares available for issuance under the 2021 Plan and 2023 Plan	2,293,816	1,000,519
Shares available for issuance under the 2023 ESPP	238,600	—
Total shares of common stock reserved for future issuance	<u>4,948,156</u>	<u>16,476,313</u>

On March 11, 2024, the Company entered into a common stock purchase agreement to issue and sell an aggregate of 6,278,905 shares of its common stock at a price of \$15.13 per share, in a private placement. The Company anticipates the gross proceeds from the private placement to be approximately \$95.0 million, before deducting any offering related expenses. The private placement financing is expected to close on March 13, 2024, subject to customary closing conditions (see Note 14).

9. Stock-based Compensation

In February 2021, the Company adopted the 2021 Equity Incentive Plan (the "2021 Plan") for the issuance of stock options to the Company's key directors, officers, employees and consultants, as a means to secure the benefits arising from capital stock ownership. In connection with the Company's IPO in November 2023, the Company adopted the 2023 Equity Incentive Plan (the "2023 Plan") and the 2023 Employee Stock Purchase Plan (the "2023 ESPP" and collectively with the 2021 Plan and 2023 Plan, the "Plans"). The purposes of the Plans are to promote the alignment of the interests of key directors, officers, employees, and consultants with the success of the Company and to provide compensation opportunities to attract, retain and motivate directors, officers, employees, and consultants of the Company.

The Company's Board of Directors adopted the 2023 Plan and the 2023 ESPP in October 2023, and the Company's stockholders approved the 2023 Plan and the 2023 ESPP in October 2023. In connection with the IPO, the Company's 2023 Equity Incentive Plan (the "2023 Plan") and its 2023 Employee Stock Purchase Plan (the "2023 ESPP") became effective. Upon the effectiveness of the 2023 Plan and the 2023 ESPP:

- 582,699 shares of common stock reserved for future issuance under the 2021 Plan ceased to be available for issuance and were added to, and became available for issuance under, the 2023 Plan, and no further grants will be made under the 2021 Plan;

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- 1,803,980 shares of common stock were reserved for future issuance under the 2023 Plan, in addition to the shares of common stock reserved for issuance under the 2021 Plan that were added to the shares reserved under the 2023 Plan; and
- 238,600 shares of common stock were reserved for future issuance under the 2023 ESPP.

The maximum number of shares of common stock that may be issued under the 2023 Plan is 4,737,000 shares, which is approximately the sum of (i) 1,803,980 new shares, plus (ii) the 2021 Plan's available reserve, plus (iii) the number of returning shares, if any, upon the cancellation or forfeiture of equity awards that are outstanding under the 2021 Plan. In addition, the number of shares of common stock reserved for issuance under the 2023 Plan will automatically increase on January 1 of each year, beginning on January 1, 2024, and continuing through and including January 1, 2033, by 5% of the total number of shares of common stock outstanding on December 31 of the immediately preceding calendar year, or a lesser number of shares determined by the Company's Board of Directors prior to the applicable January 1. The number of shares of common stock reserved for issuance under the 2023 ESPP will automatically increase on January 1 of each calendar year, beginning on January 1, 2024 and continuing through and including January 1, 2033, by the lesser of (i) 1% of the total number of shares of capital stock outstanding on December 31 of the preceding calendar year, (ii) 477,200 shares and (iii) a number of shares determined by the Company's Board of Directors. Shares subject to purchase rights granted under the 2023 ESPP that terminate without having been exercised in full will not reduce the number of shares available for issuance under the 2023 ESPP.

As of December 31, 2023, 2,293,816 shares and 238,600 shares were available for future issuance under the 2023 Plan and 2023 ESPP, respectively. No shares have been issued under the 2023 ESPP through December 31, 2023.

Stock option activity

Stock options granted under the 2021 Plan generally (i) are subject to requisite service requirements, (ii) vest over a four-year period with 25% of the options granted vesting after one year and the remainder vesting in equal monthly installments over the following 36 months, and (iii) allow for early exercise subject to repurchase. Stock options granted under the 2021 Plan to certain of the Company's non-employees vest in equal monthly installments over a four-year period or vested upon the achievement of a certain milestone event. The Company has not repurchased any shares of common stock issued pursuant to the early exercise of stock options granted under the 2021 Plan through December 31, 2023.

Stock options granted under the 2023 Plan generally (i) are subject to requisite service requirements, and (ii) vest over a four-year period with 25% of the options granted vesting after one year and the remainder vesting in equal monthly installments over the following 36 months.

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The following table summarizes the stock option activity under the 2021 Plan and the 2023 Plan for the years ended December 31, 2023 and December 31, 2022 (weighted-average remaining contractual term (in years) is not stated in thousands):

	Number of Shares	Weighted-Average Exercise Price	Weighted-Average Grant Date Fair Value	Weighted-Average Remaining Contractual Term (in years)	Aggregate Intrinsic Value
Outstanding as of December 31, 2022	1,916,194	\$ 4.64	\$ 3.00	8.65	\$20,130
Granted	1,227,954	13.63	9.89		
Exercised	(11,916)	4.27	2.75		—
Forfeited	(500,542)	9.36	6.67		
Expired	(215,950)	3.73	2.38		
Outstanding as of December 31, 2023	<u>2,415,740</u>	<u>\$ 8.32</u>	<u>\$ 5.80</u>	<u>7.83</u>	<u>\$13,689</u>
Vested options outstanding and exercisable as of December 31, 2022	661,239	\$ 3.75	\$ 2.39	8.55	\$ 7,540
Unvested options outstanding and exercisable as of December 31, 2022	1,254,955	\$ 5.12	\$ 3.32	8.70	\$12,590
Vested options outstanding and exercisable as of December 31, 2023	933,200	\$ 5.68	\$ 3.53	6.78	\$ 7,552
Unvested options outstanding and exercisable as of December 31, 2023	1,482,540	\$ 9.98	\$ 7.22	8.49	\$ 6,137

The aggregate intrinsic value of stock options is calculated as the difference between the exercise price of the stock options and the fair value of the Company's common stock for those stock options that had exercise prices lower than the fair value of the Company's common stock.

The total grant date fair values of options vested during the years ended December 31, 2023 and December 31, 2022 was \$2.4 million and \$1.6 million, respectively, including \$0.1 million for a total of 342,404 stock options granted to certain nonemployees that vested upon the achievement of a certain milestone event during the year ended December 31, 2022.

The Company estimated the fair value of options granted using a Black-Scholes option pricing model with the following assumptions presented on a weighted average basis (not stated in thousands):

	Year Ended December 31,	
	2023	2022
Risk-free interest rate	3.43%	2.76%
Expected term (in years)	5.78	6.03
Expected volatility	67.59%	73.36%
Expected dividend yield	0.00%	0.00%

The expected dividend yields are 0.00% as the Company has never paid cash dividends and does not expect to pay any cash dividends in the foreseeable future.

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Stock-based compensation expense

Stock-based compensation expense was classified as follows in the Company's consolidated statements of operations and comprehensive loss:

	Year Ended December 31,	
	2023	2022
Research and development expense	\$1,751	\$ 966
General and administrative expense	1,292	815
Total stock-based compensation expense	<u>\$3,043</u>	<u>\$1,781</u>

As of December 31, 2023 there was \$9.8 million of unrecognized stock-based compensation expense related to unvested stock options estimated to be recognized over a weighted-average period of 2.97 years.

10. Net Loss per Share

Basic and diluted net loss per common share attributable to common stockholders was calculated as follows:

	Year Ended December 31,	
	2023	2022
Numerator:		
Net loss attributable to common stockholders	\$ (66,394)	\$ (59,277)
Denominator:		
Weighted-average common shares outstanding, basic and diluted	5,354,368	1,630,348
Net loss per share attributable to common stockholders, basic and diluted	<u>\$ (12.40)</u>	<u>\$ (36.36)</u>

11. Income Taxes

A reconciliation of the Company's statutory income tax rate to the Company's effective income tax rate is as follows for the years ended December 31, 2023, and 2022:

	Year Ended December 31,	
	2023	2022
Federal statutory rate	21.00%	21.00%
State Taxes, net of federal benefit	10.80	11.77
Change in valuation allowance	(36.12)	(36.43)
Permanent differences	(0.36)	0.08
Tax credits	4.80	3.66
Other	(0.12)	(0.08)
Effective income tax rate	<u>0.00%</u>	<u>0.00%</u>

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The principal components of the Company's deferred tax assets and liabilities at December 31, 2023 and 2022 are as follows:

	Year Ended December 31,	
	2023	2022
Deferred tax assets:		
Net operating loss carryforwards	\$ 21,904	\$ 15,379
Tax credits	7,070	3,883
Intangibles	4,241	3,407
Capitalized R&D expenses	25,127	13,823
Lease liabilities	3,244	4,423
Other deferred tax assets	3,753	1,309
Total deferred tax assets	65,339	42,224
Valuation allowance	(61,974)	(37,992)
Net deferred tax assets	\$ 3,365	\$ 4,232
	Year Ended December 31,	
	2023	2022
Deferred tax liabilities:		
Right of use asset	\$(3,102)	\$(4,214)
Other deferred tax liabilities	(263)	(18)
Total deferred tax liabilities	(3,365)	(4,232)
Net deferred tax assets (liabilities)	\$ —	\$ —

As of December 31, 2023 and 2022, the Company had federal net operating loss carryforwards of approximately \$70.2 million and \$48.6 million, respectively, which can be carried forward indefinitely. As of December 31, 2023 and 2022, the Company had state and local net operating loss carryforwards of approximately \$139.3 million and \$109.3 million, respectively, which begin to expire in 2040.

As of December 31, 2023 and 2022, the Company had federal tax credits of \$7.1 and \$3.9 million, respectively, which begin to expire in 2041.

Future realization of the tax benefits of existing temporary differences and net operating loss carryforwards ultimately depends on the existence of sufficient taxable income within the carryforward period. As of December 31, 2023 and 2022, the Company performed an evaluation to determine whether a valuation allowance was needed. The Company considered all available evidence, both positive and negative, which included the results of operations for the current and preceding years. The Company determined that it was not possible to reasonably quantify future taxable income and determined that it is more likely than not that all of the deferred tax assets will not be realized. Accordingly, the Company maintained a full valuation allowance as of December 31, 2023 and 2022.

Under Internal Revenue Code Section 382, if a corporation undergoes an "ownership change," the corporation's ability to use its pre-change net operating loss ("NOL") carryforwards and other pre-change tax attributes to offset its post-change income may be limited. The Company has not completed a study to assess whether an "ownership change" has occurred or whether there have been multiple ownership changes since it became a "loss corporation" as defined in Section 382. Future changes in the Company's stock ownership, which may be outside of its control, may trigger an "ownership change." In addition, future equity offerings or acquisitions that have equity as a component of the purchase price could result in an "ownership change." If an "ownership change" has

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occurred or does occur in the future, utilization of the NOL carryforwards or other tax attributes may be limited, which could potentially result in increased future tax liability to the Company.

The calculation of the Company's tax liabilities involves dealing with uncertainties in the application of complex tax laws and regulations for both federal taxes and the many states in which it operates or does business in. ASC 740 states that a tax benefit from an uncertain tax position may be recognized when it is more likely than not that the position will be sustained upon examination, including resolutions of any related appeals or litigation processes, on the basis of the technical merits.

The Company records uncertain tax positions as liabilities in accordance with ASC 740 and adjusts these liabilities when its judgment changes as a result of the evaluation of new information not previously available. Because of the complexity of some of these uncertainties, the ultimate resolution may result in a payment that is materially different from our current estimate of the unrecognized tax benefit liabilities. These differences will be reflected as increases or decreases to income tax expense in the period in which new information is available. As of December 31, 2023 and 2022, the Company has not recorded any uncertain tax positions in its consolidated financial statements.

The Company recognizes interest and penalties related to uncertain tax positions on the income tax expense line in the accompanying consolidated statements of operations. As of December 31, 2023 and 2022, no accrued interest or penalties are included on the related tax liability line in the consolidated balance sheet.

The Company files tax returns as prescribed by the tax laws of the jurisdictions in which it operates. In the normal course of business, the Company is subject to examination by federal and state jurisdictions, where applicable. The resolution of tax matters is not expected to have a material effect on the Company's consolidated financial statements.

12. Commitments and Contingencies

Leases—As of December 31, 2023, the Company had entered into commitments under lease agreements to rent office space and finance equipment (see Note 5).

Commitments—As of December 31, 2023, the Company had entered into commitments under license, acquisition, research collaboration and sponsored research agreements with third parties (see Note 13). In addition, the Company has entered into services agreements with third parties for pharmaceutical manufacturing and research activities in the normal course of business, which can generally be terminated by the Company with 30- to 60-days' written notice, unless otherwise indicated. Further, certain of the Company's manufacturing agreements could require early termination and wind-down payments due from the Company upon either the termination of its clinical trials or if the Company terminates such agreements for convenience.

Contingencies—From time to time, the Company may have certain contingent liabilities that arise in the ordinary course of business. The Company recognizes any associated legal fees as incurred and accrues a liability for such contingent liability matters when it is probable that future expenditures will be made, and such expenditures can be reasonably estimated. For all periods presented, the Company was not a party to any pending material litigation or other material legal proceedings, except that on October 12, 2023, Rocket Pharmaceuticals, Inc. ("Rocket") filed a lawsuit in the U.S. District Court for the Southern District of New York against the Company and two individuals claiming, among other things, misappropriation of confidential information and trade secrets. The individual defendants are a current employee and a former employee of the Company's analytical development team, both of whom were employed at Rocket before joining the Company in 2021. The complaint alleges the individual defendants downloaded confidential Rocket company documents and other proprietary materials prior to leaving Rocket in 2021 and that the Company used this information to advance its programs. The complaint seeks unspecified damages and asks the court to enjoin the Company from competing and working in the market for gene therapy treatments targeting cardiac diseases. The Company retained legal counsel to assist with its ongoing review of the allegations in Rocket's complaint and is confident in its defenses.

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to the allegations. On December 7, 2023, the Company filed a motion to dismiss the complaint, which is now fully briefed and pending before the court. While it is not possible to predict the outcome with certainty and an estimate of the possible loss cannot be made, the Company currently does not expect the final outcome will have a material adverse effect on its timelines for development of its product candidates.

Indemnification Agreements—In the ordinary course of business, the Company may provide indemnification of varying scope and terms to vendors, lessors, business partners and other parties with respect to certain matters including, but not limited to, losses arising out of breach of such agreements or from intellectual property infringement claims made by third parties. In addition, the Company has entered into indemnification agreements with members of its Board of Directors and executive officers that will require the Company, among other things, to indemnify them against certain liabilities that may arise by reason of their status or service as directors or officers. The maximum potential amount of future payments the Company could be required to make under these indemnification agreements is, in many cases, unlimited. For all periods presented, the Company has not incurred any material costs as a result of such indemnifications.

13. License, Acquisition, Research and Collaboration and Sponsored Research Agreements

Adverum Biotechnologies—On January 25, 2021, the Company entered into an exclusive license agreement with Adverum Biotechnologies Inc. (“Adverum”) to in-license materials and technology related to the treatment of cardiomyopathy due to Friedreich’s Ataxia (the “Adverum Agreement”). In connection with the Adverum Agreement, the Company gained access to a portfolio of inventions, patent rights, technology, and licensed methods that the Company continues to develop, and the Company will assume all development and commercialization activities worldwide. Pursuant to the Adverum Agreement, the Company paid a one-time up-front non-refundable fee of \$7.5 million, and is obligated to pay aggregate development and regulatory milestones of up to \$17.5 million including a \$3.5 million development milestone that was achieved and paid in the first quarter of 2023, and aggregate sales event and commercialization milestones of up to \$49.0 million. The Company is obligated to pay Adverum tiered royalties ranging from high single-digits to sub teens based on annual aggregate worldwide net sales of Products (as defined in the Adverum Agreement). As of December 31, 2023, there were no research and development expenses recorded by the Company or payments made to Adverum under the terms of the Adverum Agreement other than the one-time up-front non-refundable fee of \$7.5 million and the \$3.5 million development milestone that was achieved and paid in the first quarter of 2023.

The Adverum Agreement remains in effect until termination at the date of the last royalty term to expire. The Company can terminate the Adverum Agreement with 120 days’ written notice. The Adverum Agreement can also be terminated as a result of a patent challenge, material breach of contractual terms, or insolvency by either party.

Cornell University—On May 27, 2020, the Company entered into two exclusive license agreements with Cornell University (“Cornell”) (the “Cornell First License Agreement” and the “Cornell Second License Agreement,” collectively “the Cornell License Agreements”). The Cornell First License Agreement is for the in-license of technology related to portfolios for APOE-associated Alzheimer’s disease and Anti-Tau, although the Company’s license is not restricted by such indications and it includes assignment to the Company of Cornell’s IND for the use of AAVrh10.hAPOE2 vector to treat APOE4 homozygous patients who are at risk for or have Alzheimer’s disease to support development of the Company’s LX1001 program. The Cornell Second License Agreement is for the in-license of technology related to a portfolio for Friedreich’s ataxia although the Company’s license is not restricted by such indications. Through the Cornell License Agreements, the Company gains access to a portfolio of inventions, patent rights, technology, and licensed methods that the Company continues to develop. Under the terms of the Cornell License Agreements, the Company has assumed all development and commercialization activities worldwide with respect to the licensed technology.

As initial consideration for the Cornell License Agreements, the Company paid Cornell an upfront payment in cash of \$0.3 million and issued \$1.3 million of notes (the “Notes”). In November 2020, the Notes with outstanding principal of \$1.3 million were cancelled in exchange for 1,337,610 shares of series A convertible

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preferred stock. As additional consideration, the Company is required to pay Cornell up to \$8.4 million upon the achievement of specific clinical and regulatory milestones under the Cornell First License Agreement and up to \$4.3 million in two portfolios and up to \$0.6 million for a third portfolio upon the achievement of specific clinical and regulatory milestones under the Cornell Second License Agreement. In the second quarter of 2022, a clinical and regulatory milestone of \$0.1 million was recognized and paid to Cornell in connection with the Cornell Second License Agreement. The Company is also required to pay Cornell a flat royalty in the mid-single-digits based on net sales of the products covered by the licenses, subject to certain adjustments.

Upon expiration of the royalty term of a given licensed product in a country, the respective license becomes non-exclusive and royalty-free. In addition, each of the Cornell License Agreements may be terminated by the Company for any reason upon ninety (90) days' advance notice to Cornell and by Cornell upon the Company's material uncured breach, and all licenses and rights granted by either party under such agreement will concurrently terminate.

During the year ended December 31, 2022, the Company incurred and paid \$0.1 million and \$0.1 million, respectively, of research and development expenses to Cornell in connection with the Cornell License Agreements. During the year ended December 31, 2023, the Company did not incur any research and development expenses or make any payments to Cornell in connection with the Cornell License Agreements.

Stelios Therapeutics, Inc.—Stelios was an early-stage company developing novel adeno-associated AAV-based gene therapies for rare cardiac conditions including arrhythmogenic cardiomyopathy and TNNI3-associated hypertrophic cardiomyopathy. On July 16, 2021, the Company acquired 100% of the outstanding stock of Stelios that was accounted for as an asset acquisition pursuant ASC 805, *Business Combinations*. The Company is required to pay up to an aggregate of \$20.5 million to the selling shareholders of Stelios upon the achievement of certain development milestones, including a \$2.0 million development milestone that was achieved and paid in the third quarter of 2022.

Regents of the University of California, San Diego—Stelios entered into exclusive worldwide license agreements on April 23, 2020, and August 6, 2020 (the "First UCSD Agreement" and the "Second UCSD Agreement", respectively) with the Regents of UCSD to in-license materials and intellectual property related to gene therapies for arrhythmogenic right ventricular cardiomyopathy and hypertrophic cardiomyopathy, respectively. The First UCSD Agreement and the Second UCSD Agreement relate to the Company's development efforts for its LX2021 and LX2022 programs, respectively. In connection with the First UCSD Agreement and the Second UCSD Agreement, the Company gained access to inventions, patent rights, technology, and licensed methods that it continues to develop, and it has assumed all worldwide development and commercialization activities with respect to the licensed technologies. The First UCSD Agreement and Second UCSD Agreement required Stelios to pay one-time up-front non-refundable cash fees of \$20,000 for each agreement and requires the Company to pay aggregate development and commercialization milestones of up to \$4.8 million and \$2.4 million, respectively, and low- to mid-single digit royalties and low-single digit royalties, respectively, based on aggregate net sales. The only research and development expenses incurred by Stelios or the Company and payments made to the Regents of UCSD through December 31, 2023 under the terms of the First UCSD Agreement and the Second UCSD Agreement were the one-time up-front non-refundable cash fees of \$20,000 for each agreement. The Company has the right to terminate the First UCSD Agreement and the Second UCSD Agreement at any time upon sixty (60)-days' written notice to the Regents of UCSD.

On October 4, 2021, the Company entered into an exclusive worldwide license agreement (the "Third UCSD Agreement" and collectively with the First UCSD Agreement and the Second UCSD Agreement, the "UCSD Agreements") with the Regents of UCSD to in-license materials and intellectual property related to LX2020, a gene therapy for arrhythmogenic right ventricular cardiomyopathy. In connection with the Third UCSD Agreement, the Company gained access to inventions, patent rights, technology, and licensed methods that it continues to develop, and it has assumed all worldwide development and commercialization activities with respect to the licensed technology. The Third UCSD Agreement required the Company to pay a one-time up-front non-refundable cash fee of \$20,000 and requires the Company to pay aggregate development and

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commercialization milestones of up to \$4.0 million, and low-to mid-single digit royalties based on aggregate net sales. The only research and development expenses incurred by the Company and payments made to the Regents of UCSD under the terms of the Third UCSD Agreement were the one-time up-front non-refundable cash fee of \$20,000. The Company has the right to terminate the Third UCSD Agreement at any time upon sixty (60)-days' written notice to the Regents of UCSD.

On December 3, 2021, the Company entered into two sponsored research agreements with the Regents of UCSD (collectively, the "UCSD SRAs") for the Company's LX2020, LX2021 and LX2022 programs in connection with the UCSD Agreements. Under the terms of the UCSD SRAs, the Company has the first rights to obtain non-exclusive or exclusive, sublicensable, royalty-bearing, perpetual and transferable worldwide licenses in any resulting inventions owned by the Regents of UCSD or resulting jointly owned inventions between the Company and the Regents of UCSD, and the Company retains the rights to any resulting inventions owned by the Company. The UCSD SRAs each have a two-year term and may be terminated early by the Company at any time upon the giving of thirty (30) days' written notice to the Regents of UCSD. The total costs to be invoiced to the Company over the terms of the UCSD SRAs are \$5.6 million, of which \$1.6 million and \$2.3 million were recorded in research and development expenses during the years ended December 31, 2023 and December 31, 2022, respectively. The Company made payments totaling \$0.4 million and \$2.8 million to the Regents of UCSD during the years ended December 31, 2023 and December 31, 2022, respectively. The Company has paid a cumulative total of \$3.2 million to the Regents of UCSD as of December 31, 2023, in connection with the UCSD SRAs.

Weill Cornell Medical College—On February 2, 2021, the Company entered into a Research Collaboration Agreement with Weill Cornell Medical College ("WCM" and the "WCM Agreement") in conjunction with the Cornell License Agreements entered on May 27, 2020. The Company committed to fund scientific research at WCM to investigate further and potentially enhance the technology licensed to the Company pursuant to the License Agreements.

Under the terms of the WCM Agreement, each WCM invention, joint invention, and related joint results for which an Improvement, as defined in the WCM Agreement, applies and the Company has made an election to amend the Cornell License Agreements, the Company has the first option to negotiate in good faith with WCM for royalty-bearing, worldwide license, under Cornell patent rights, Cornell rights, and Cornell's interest in joint patent rights, to develop, make, have made, use, offer for sale, sell, have sold, and import derived products in the field. During the years ended December 31, 2023 and December 31, 2022 the Company incurred \$1.0 million and \$6.1 million, respectively, of research and development costs in connection with the WCM Agreement. During the years ended December 31, 2023 and December 31, 2022 the Company paid \$2.1 million and \$5.5 million, respectively, to WCM in connection with the WCM Agreement. Cumulatively, the Company has incurred and paid total research and development costs of \$9.9 to WCM in connection with the WCM Agreement as of December 31, 2023.

The WCM Agreement expired in accordance with its terms in January 2024.

14. Subsequent Events

Subsequent events have been evaluated through March 11, 2024, which is the date that these consolidated financial statements were issued and were available to be issued. On March 11, 2024, the Company entered into a common stock purchase agreement to issue and sell an aggregate of 6,278,905 shares of its common stock at a price of \$15.13 per share, in a private placement. The Company anticipates the gross proceeds from the private placement to be approximately \$95.0 million, before deducting any offering related expenses. The private placement financing is expected to close on March 13, 2024, subject to customary closing conditions.

6,974,248 Shares of Common Stock



PROSPECTUS

April 12, 2024

You should rely only on the information contained in this prospectus or any supplement or amendment hereto. We have not authorized anyone to provide you with different information. You should not assume that the information contained in this prospectus or any supplement or amendment hereto is accurate as of any date other than the date of this prospectus or any such supplement or amendment. Neither the Securities and Exchange Commission nor any state securities commission has approved or disapproved of these securities or determined if this prospectus is truthful or complete. Any representation to the contrary is a criminal offense.
