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As submitted to the Securities and Exchange Commission confidentially on February 4, 2022.
This draft registration statement has not been publicly filed with the Securities and Exchange Commission and all information herein remains strictly confidential.
Registration No. 333-

UNITED STATES
SECURITIES AND EXCHANGE COMMISSION
Washington, D.C. 20549

FORM S-1
REGISTRATION STATEMENT
UNDER
THE SECURITIES ACT OF 1933

Lexeo Therapeutics, Inc.
(Exact name of registrant as specified in its charter)

Delaware	**2836**	**85-4012572**
(State or other jurisdiction of incorporation or organization)	**(Primary Standard Industrial Classification Code Number)**	**(I.R.S. Employer Identification No.)**

430 East 29th Street, Floor 14
New York, New York 10016
(212) 547-9879
(Address, including zip code, and telephone number, including area code, of registrant's principal executive offices)

R. Nolan Townsend
Chief Executive Officer
Lexeo Therapeutics, Inc.
430 East 29th Street, Floor 14
New York, New York 10016
(212) 547-9879
(Name, address, including zip code, and telephone number, including area code, of agent for service)

Copies to:

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New York, New York 10001
(212) 479-6000

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Goodwin Procter LLP
620 Eighth Avenue
New York, New York 10018
(212) 813-8800

Approximate date of commencement of proposed sale to the public: As soon as practicable after this Registration Statement is declared effective.

If any of the securities being registered on this Form are to be offered on a delayed or continuous basis pursuant to Rule 415 under the Securities Act of 1933, check the following box. ☐

If this Form is filed to register additional securities for an offering pursuant to Rule 462(b) under the Securities Act, please check the following box and list the Securities Act registration statement number of the earlier effective registration statement for the same offering. ☐

If this Form is a post-effective amendment filed pursuant to Rule 462(c) under the Securities Act, check the following box and list the Securities Act registration statement number of the earlier effective registration statement for the same offering. ☐

If this Form is a post-effective amendment filed pursuant to Rule 462(d) under the Securities Act, check the following box and list the Securities Act registration statement number of the earlier effective registration statement for the same offering. ☐

Indicate by check mark whether the registrant is a large accelerated filer, an accelerated filer, a non-accelerated filer, smaller reporting company, or an emerging growth company. See the definitions of "large accelerated filer," "accelerated filer," "smaller reporting company," and "emerging growth company" in Rule 12b-2 of the Exchange Act.

Large accelerated filer	☐	Accelerated filer	☐
Non-accelerated filer	☒	Smaller reporting company	☒
		Emerging growth company	☒

If an emerging growth company, indicate by check mark if the registrant has elected not to use the extended transition period for complying with any new or revised financial accounting standards provided pursuant to Section 7(a)(2)(B) of the Securities Act. ☐

The Registrant hereby amends this Registration Statement on such date or dates as may be necessary to delay its effective date until the Registrant shall file a further amendment which specifically states that this Registration Statement shall thereafter become effective in accordance with Section 8(a) of the Securities Act of 1933 or until the Registration Statement shall become effective on such date as the Securities and Exchange Commission, acting pursuant to said Section 8(a), may determine.

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The information in this preliminary prospectus is not complete and may be changed. These securities may not be sold until the registration statement filed with the Securities and Exchange Commission is effective. This preliminary prospectus is not an offer to sell these securities nor does it seek an offer to buy these securities in any jurisdiction where the offer or sale is not permitted.

Subject to completion, dated , 2022

PRELIMINARY PROSPECTUS

Shares

Common Stock

This is an initial public offering of shares of common stock of Lexeo Therapeutics, Inc.

We are offering shares of our common stock. The initial public offering is expected to be between $ and $ per share.

Prior to this offering, there has been no public market for our common stock. We intend to apply to list our common stock on the Nasdaq Global Market under the trading symbol "LXEO."

We are an "emerging growth company" and a "smaller reporting company" as defined under U.S. federal securities laws and, as such, will be subject to reduced public company reporting requirements for this prospectus and future filings. See "Prospectus Summary—Implications of Being an Emerging Growth Company and a Smaller Reporting Company."

Investing in our common stock involves risks. See "Risk Factors" beginning on page 14 to read about factors you should consider before buying shares of our common stock.

Neither the Securities and Exchange Commission nor any state securities commission has approved or disapproved of these securities, or passed upon the accuracy or adequacy of this prospectus. Any representation to the contrary is a criminal offense.

	Per Share	Total
Initial public offering price	$	$
Underwriting discounts (1)	$	$
Proceeds, before expenses, to Lexeo Therapeutics, Inc.	$	$

(1) See the section titled "Underwriting" for a description of the compensation payable to the underwriters.

We have granted the underwriters an option for a period of 30 days to purchase up to additional shares from us at the public offering price less the underwriting discount.

The underwriters expect to deliver the shares against payment in New York, New York on , 2022.

J.P. Morgan **SVB Leerink** **Stifel** **RBC Capital Markets**

Chardan

Prospectus dated , 2022.

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Neither we nor the underwriters have authorized anyone to provide any information or to make any representations other than those contained in this prospectus or in any free writing prospectuses we have prepared. We and the underwriters take no responsibility for, and can provide no assurance as to the reliability of, any other information that others may provide you. We are offering to sell, and seeking offers to buy, shares of common stock only in jurisdictions where offers and sales are permitted. The information contained in this prospectus is accurate only as of the date of this prospectus, regardless of the time of delivery of this prospectus or of any sale of the common stock.

For investors outside of the United States: We have not, and the underwriters have not, done anything that would permit this offering or possession or distribution of this prospectus in any jurisdiction where action for that purpose is required, other than the United States. Persons outside of the United States who come into possession of this prospectus must inform themselves about, and observe any restrictions relating to, the offering of the shares of common stock and the distribution of this prospectus outside of the United States.

All trademarks, trade names and service marks appearing in this prospectus are the property of their respective owners. Solely for convenience, the trademarks and trade names in this prospectus may be referred to without the ® and ™ symbols, but such references should not be construed as any indicator that their respective owners will not assert their rights thereto.

Implications of the FAST Act

Pursuant to the applicable provisions of the Fixing America's Surface Transportation Act, we are not required to file our financial information for the year ended December 31, 2019 because we expect to file our financial information for the year ended December 31, 2021 in our registration statement when it is first publicly filed. While the financial information for the year ended December 31, 2019 is otherwise required by Regulation S-X, it will not be required to be included in the Form S-1 filing at the time of the contemplated offering.

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," "Lexeo" and "Lexeo Therapeutics" refer to Lexeo Therapeutics, Inc.

Overview

We are a clinical-stage gene therapy company focused on addressing some of the most devastating genetically defined cardiovascular and central nervous system diseases affecting both larger-rare and prevalent patient populations. We are advancing a deep and diverse pipeline of AAV-based gene therapy candidates utilizing our modular approach that integrates clinically validated technology, a disease area strategy targeting defined patient sub-populations most likely to benefit from our gene therapy candidates, and high-quality, scalable manufacturing, which is designed to overcome many of the challenges facing the field of gene therapy.

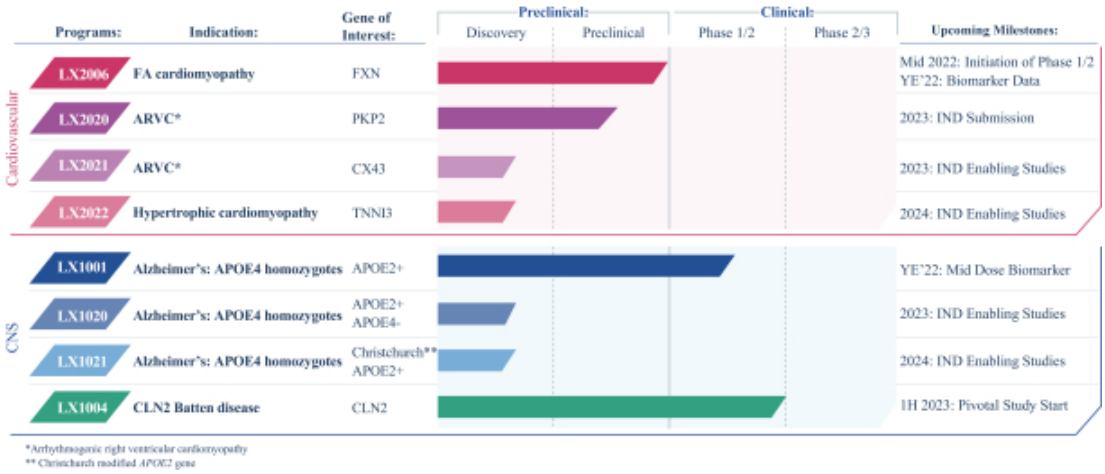
We expect to initiate a Phase 1/2 clinical trial in mid-2022 for our most advanced cardiovascular program, LX2006 for the treatment of patients with the cardiac manifestation of Friedreich's ataxia, or FA cardiomyopathy, and we expect to report initial biomarker data by the end of 2022. Our lead central nervous system, or CNS, product candidate, LX1001, is in an ongoing Phase 1/2 trial for the treatment of apolipoprotein E4, or APOE4, associated Alzheimer's disease. We have initially observed an increase in expression levels of the protective protein, APOE2, and a reduction in core Alzheimer's disease biomarkers in the low dose cohort of this ongoing trial. We expect to report initial biomarker data from the mid-dose cohort by the end of 2022. Utilizing a step-wise, capital-efficient development approach, we are leveraging early proof-of-concept functional and biomarker data to build a pipeline of gene therapy programs targeting cardiovascular indications and Alzheimer's disease for genetically defined populations.

Our integrated, modular approach enables us to optimize our strategy to pursue larger-rare and prevalent genetically defined indications in specific sub-populations 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 and safety profile for our product candidate for each disease. Our most advanced cardiovascular and CNS programs use the AAVrh10 vector due to its high transduction efficiency in both myocardial cells and neurons, potentially lower toxicity given its ability to utilize lower doses compared to other well-established adeno-associated virus, or AAV, serotypes, and lower pre-existing immunity.

By specifically tailoring our technological approach to each targeted disease, we believe we can design our programs to achieve the highest likelihood of having therapeutic impact. We target genetically defined indications in specific sub-populations of patients that may be most amenable to gene therapy. These target indications offer the potential to show therapeutic impact through functional endpoints or biomarkers, have high unmet need and large market opportunities, have demonstrated promising preclinical data, and have organized patient advocacy groups and identifiable patient populations. We believe targeting cardiac and CNS diseases can be enhanced by our current approach utilizing AAVrh10 as well as ongoing discovery efforts to identify a next-generation vector technology with the best potential therapeutic profile. Finally, we continuously seek to bolster our pipeline through relationships with academic institutions, which provide us access to cutting edge gene therapy research that we will utilize in the discovery and development of next generation gene therapy candidates.

Our pipeline

We are advancing a deep and diverse pipeline of cardiovascular and CNS therapeutic programs for larger-rare and prevalent diseases. We retain exclusive worldwide development and commercialization rights to all of our product candidates and programs.



Lead cardiovascular programs

We are seeking to develop a number of disease-modifying gene therapy candidates to treat larger-rare cardiovascular diseases that have significant unmet need and no approved treatments addressing 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 Friedreich's ataxia and affects approximately 5,600 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 as well as restoration of cardiac function and reversal of the disease abnormalities of FA cardiomyopathy in a partial *FXN* knockout mouse model. Our investigational new drug application, or IND, for LX2006 was cleared by the U.S. Food and Drug Administration, or FDA, in , 2022. We expect to initiate an open-label, dose-escalation Phase 1/2 clinical trial in patients with FA cardiomyopathy in mid-2022, and we expect to report interim biomarker data by the end of 2022. The FDA has granted Rare Pediatric Disease designation and Orphan Drug designation to LX2006 for the treatment of Friedreich's ataxia.
- *LX2020* is an AAVrh10-based gene therapy candidate designed to intravenously deliver a fully functional *PKP2* gene to cardiac muscle for the treatment of arrhythmogenic right ventricular cardiomyopathy, or ARVC, caused by mutations in the *PKP2* gene. *PKP2* mutations are associated with approximately 75% of all genetic cases of ARVC, and we estimate they affect more than 70,000 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, LX2020 resulted in fewer arrhythmias and increased survival. We intend to submit an IND for LX2020 in 2023.

Lead CNS programs

We are developing a pipeline of CNS focused gene therapies that includes a portfolio of approaches to treat the genetics underlying Alzheimer's disease as well as a program designed to treat neuronal ceroid lipofuscinosis type 2 (CLN2) disease, or CLN2 Batten disease.

- *LX1001* is an AAVrh10-based gene therapy candidate designed to deliver via one-time administration 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 CNS. *APOE4* homozygous individuals 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. Currently, there are no approved disease-modifying treatments for Alzheimer's disease. 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 and we have observed a decline in Alzheimer's disease CSF biomarkers, such as total tau and phosphorylated tau, in the first two patients with 12-month data in the low-dose cohort in the trial. We have also reported data demonstrating expression of the protective APOE2 protein in all four patients in the low-dose cohort with follow-up data. We expect to report initial biomarker data from the mid-dose cohort by the end of 2022. LX1001 has been granted fast track designation by the FDA.
- *LX1004* is an AAVrh10-based gene therapy candidate designed to deliver via intracisternal injection a fully functional *CLN2* gene to the CNS in order to restore the enzyme tripeptidyl peptidase 1, or TPP1, the secreted protein that is deficient in CLN2 Batten disease patients. This disease is an autosomal recessive lysosomal storage disorder causing loss of cognitive and motor function, blindness, seizures, and ultimately death in childhood, with approximately 900 cases estimated in the United States and European Union. In a completed Phase 1/2 clinical trial, LX1004 was administered via intraparenchymal injection and demonstrated an increase in TPP1 levels leading to a slower decline of motor and language function at 18 months post treatment in treated patients compared to natural history controls. We anticipate receiving feedback from the FDA on the design of our potentially pivotal Phase 2/3 clinical trial in the second half of 2022, and we intend to initiate the clinical trial in the first half of 2023. The FDA has granted Rare Pediatric Disease designation and Orphan Drug designation to LX1004 for the treatment of CLN2 Batten disease.

Additional programs

Within our cardiovascular pipeline, we are concurrently advancing several additional AAV-based gene therapy programs to treat other targets in myocardial cells that are dysregulated in various types of cardiomyopathy, including LX2021 to restore Connexin 43, or Cx43, in patients with ARVC, and LX2022 to treat hypertrophic cardiomyopathy, or HCM, due to mutations in the *TNNI3* gene.

We are also building a portfolio of approaches to treat the genetics underlying Alzheimer's disease. Our Alzheimer's disease portfolio includes LX1020, which is designed to deliver both the protective *APOE2* gene and microRNA, or miRNA, to suppress the expression of the deleterious *APOE4* gene, and LX1021, which is designed to deliver a Christchurch-modified *APOE2* gene. The Christchurch mutation has been shown in individuals to be effective at protecting patients against Alzheimer's disease even in the presence of significant amyloid pathology.

Our approach

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

Our technology approach

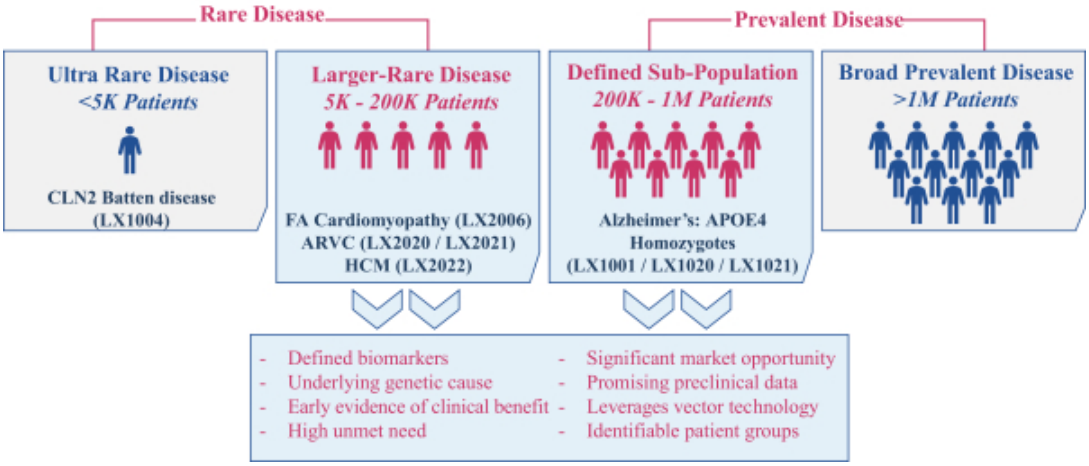
We believe that our approach to technology confers the following advantages:

- **High Transduction Efficiency and Biodistribution**. The AAVrh10 vector has proven to be effective at transducing myocardial cells and neurons, which we believe makes it optimal for delivery and expression of transgenes for the treatment of the cardiovascular and CNS diseases we are currently targeting.
- **Reduced Toxicity**. The cardiac tropism allows AAVrh10 to be systemically administered at lower doses than many other AAV-based therapies targeting cardiovascular or other systemic diseases.
- *Reduced Pre-Existing Neutralizing Antibodies.* Among the naturally occurring and commonly used AAV serotypes, AAVrh10 has been shown 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 therapeutic efficacy of our product candidates.

Our disease area strategy

We employ a strategy to select targets in indications that have relatively large patient populations, high unmet need and technically feasible treatment profiles.

Our disease area strategy



We target diseases with the following characteristics:

- *Defined sub-populations in 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, increasing the homogeneity of our studied patient populations and the likelihood of efficacy.
- *Indications with potential to demonstrate early evidence of meaningful clinical benefit.* We pursue clearly defined biomarkers, as well as functional endpoints, that can potentially provide early proof-of-mechanism and inform clinical development decisions, and may also facilitate accelerated approval pathways.
- *Present opportunity to address high unmet medical need.* We are focused on genetically defined cardiovascular and CNS conditions where there is no currently approved treatment or where we believe our therapeutic candidates will have a meaningful improvement relative to existing standards of care.
- **Significant market opportunity.** We seek indications with significant commercial opportunities beyond those typically associated with gene therapy companies targeting rare monogenic diseases. Our cardiac pipeline and our Alzheimer's disease portfolio target either larger-rare or prevalent patient populations.
- **Targets that have demonstrated promising preclinical data.** 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 or acquire product candidates with established proof of concept in relevant animal models across a wide range of indications.
- **Targeted disease areas best treated by optimal vector technologies.** Our initial gene therapy candidates utilize the AAVrh10 vector due its high transduction efficiency and biodistribution profile. For future indications, we will pursue the optimal vector technology to address the diseases of interest while ensuring sufficient preclinical or clinical validation for any novel approaches.
- **Readily identifiable patient groups.** 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 are also leveraging existing natural history studies which can help us to better define the target patient phenotypes associated with the disease.

Our manufacturing approach

We are developing gene therapy candidates for larger-rare and prevalent disease patient populations, which require a high-quality process that can produce vector in relatively large quantities while utilizing established biologics manufacturing infrastructure. We use a baculovirus/Sf9 expression system to manufacture our gene therapy candidates due to its scalability and efficiency. Our manufacturing platform infects Sf9 cells at high densities in suspension cell culture with a capsid baculovirus and a transgene baculovirus. The output is coupled with a chromatography-based purification process which allows for efficient AAV purification, resulting in higher vector yield, fewer empty AAV capsids, reduced incorporation of non-transgene DNA impurities and lower levels of process residuals than traditionally used plasmid HEK adherent cell culture approaches.

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. We

believe our ongoing work with these 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 company and team

We are led by pioneers and experts with decades of collective experience in genetic medicine, rare disease drug development, manufacturing and commercialization. Our scientific founder and Chief Scientific Advisor, Ronald G. Crystal, M.D., is a world leader in gene therapy research and development and currently serves as Professor and Chairman of Weill Cornell Medicine's Department of Genetic Medicine and Director of the Belfer Gene Therapy Core Facility. Our Chief Executive Officer, R. Nolan Townsend, has spent more than a decade working in global biopharmaceutical commercial organizations. Most recently, he led Pfizer's U.S. rare disease commercial business unit, where he oversaw the successful U.S. launch of blockbuster rare cardiovascular product (tafamidis) Vyndaqel/Vyndamax. With over 20 years in the biopharmaceutical industry, Jay A. Barth, M.D., our Executive Vice President and Chief Medical Officer, previously held leadership roles at Amicus Therapeutics, Inc. and PTC Therapeutics, Inc. and had clinical oversight of the initial approvals of Galafold, for the treatment of Fabry disease, and Translarna, the first approved treatment for Duchenne muscular dystrophy. Our Chief Technical Officer, Paul McCormac, Ph.D., has more than 20 years of experience in the field of gene therapy and biologics chemistry, manufacturing, and controls, or CMC, and previously led gene therapy CMC and vector supply strategy for Pfizer's rare disease business and research units. Our Chairman, Steven Altschuler, M.D., is currently Managing Director at Ziff Capital Investments and was formerly Chairman of the gene therapy pioneer Spark Therapeutics, Inc., which was responsible for the first FDA-approved gene therapy, voretigene neparvovec-rzyl (Luxturna), and was acquired by Roche Holding AG in 2019 for $4.3 billion.

Since our inception, we have raised $183.7 million in capital from premier institutional investors, including funds affiliated with D1 Capital Partners, Eventide Healthcare & Life Sciences Fund, Janus Henderson Investors, Longitude Capital, Lundbeckfonden Ventures, Omega Funds and PBM Capital.

Our strategy

Our vision is a world where gene therapies resolve the burden of disease, transforming the lives of patients. The key elements of our strategy to achieve this vision are to:

- Focus on genetically defined cardiovascular and CNS indications of high unmet need and substantial commercial potential.
- Target development efforts at disease pathologies and defined sub-populations of patients most likely to benefit from our gene therapy candidates.
- Pursue a step-wise and capital-efficient development approach for advancing our programs through the clinic and regulatory approval.
- Expand our cardiovascular pipeline capabilities through the discovery and development of next-generation gene therapy technologies.
- Leverage and expand upon our partnerships and exclusive licenses with world class academic institutions.
- Utilize a scalable, unified manufacturing platform across all current and future programs.
- Build a fully integrated gene therapy 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 that you should be aware of before making an investment decision. These risks are described more fully in the section titled “Risk Factors” and include, among others:

- 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.
- 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.
- Even if this offering is successful, 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.
- 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, 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 neither predict the geographic areas in which we could obtain regulatory approval nor 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.
- 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.
- 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.

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

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 $1.07 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 30th, 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 430 East 29th Street, Floor 14, New York, New York 10016, 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 prospectus.

The offering

Common stock offered by us	shares
Underwriters' option to purchase additional shares	We have granted the underwriters an option for a period of 30 days from the date of this prospectus to purchase up to additional shares of our common stock.
Common stock to be outstanding immediately after this offering	shares (or shares if the underwriters exercise in full their option to purchase additional shares).
Use of proceeds	We estimate that the net proceeds from this offering will be approximately $ million, (or approximately $ million if the underwriters exercise in full their option to purchase up to additional shares of common stock), assuming an initial public offering price of $ per share, which is the midpoint of the price range set forth on the cover page of this prospectus, after deducting the estimated underwriting discounts and commissions and estimated offering expenses payable by us. We currently intend to use the net proceeds from this offering, together with our existing cash, to advance the clinical development of LX2006, LX1001 and LX1004, to fund the development of our other product candidates and discovery efforts, and for working capital and other general corporate purposes. See the section titled "Use of Proceeds" for additional information.
Risk factors	You should read the section titled "Risk Factors" for a discussion of factors you should consider carefully, together with all the other information included in this prospectus, before deciding to invest in our common stock.
Proposed Nasdaq Global Market symbol	"LXEO"

The number of shares of our common stock to be outstanding after this offering is based on shares of our common stock outstanding as of , 2022 after giving effect to the conversion of all outstanding shares of our convertible preferred stock into an aggregate of shares of common stock, and excludes:

- shares of our common stock issuable upon the exercise of options under our 2021 Equity Incentive Plan, or the Existing Plan, granted subsequent to , 2022 at a weighted-average exercise price of $ per share;
- shares of our common stock reserved for future issuance under the Existing Plan as of , 2022, which shares will cease to be available for issuance at the time our 2022 Stock Incentive Plan, or the 2022 Plan, becomes effective and will be added to, and become available for issuance under, the 2022 Plan;

- shares of our common stock reserved for future issuance under our 2022 Plan, which will become effective in connection with this offering, as well as any automatic increases in the number of shares of common stock reserved for future issuance under the 2022 Plan; and
- shares of our common stock reserved for future issuance under our 2022 Employee Stock Purchase Plan, or the ESPP, which will become effective in connection with this offering, as well as any automatic increases in the number of shares of common stock reserved for future issuance under the ESPP.

Unless otherwise indicated, all information contained in this prospectus, including the number of shares of common stock that will be outstanding after this offering, assumes or gives effect to:

- the conversion of all outstanding shares of our preferred stock on a one-for-one basis into shares of our common stock, which will occur upon the closing of this offering;
- a -for- stock split of our common stock effected on ;
- the filing and effectiveness of our amended and restated certificate of incorporation upon the closing of this offering;
- no exercise of the outstanding options referred to above after , 2022; and
- no exercise by the underwriters of their option to purchase additional shares of our common stock.

Summary financial data

The following tables set forth our summary financial data for the years ended December 31, 2020 and 2021. We have derived the statement of operations and comprehensive loss data for the year ended December 31, 2020 and 2021 and the balance sheet as of December 31, 2021 from our audited financial statements included elsewhere in this prospectus. In the opinion of management, the unaudited data reflects all adjustments, consisting only of normal recurring adjustments, considered necessary for a fair presentation of the financial information in those statements.

You should read the following summary data together with our financial statements and the related notes thereto included elsewhere in this prospectus and the section of this prospectus titled "Management's Discussion and Analysis of Financial Condition and Results of Operations." The summary financial data in this section are not intended to replace our financial statements and are qualified in their entirety by our financial statements and related notes included elsewhere in this prospectus. Our historical results are not necessarily indicative of the results that should be expected in the future.

	Year ended December 31	
	2020	**2021**
Grant revenue	$ 518,476	
Operating expenses:		
Research and development	4,318,765	
General and administrative	787,499	
Total operating expenses	**5,106,264**	
Loss from operations	(4,587,788)	
Other expense:		
Loss on extinguishment of notes	(422,091)	
Other Income	4,241	
Interest Expense	(147,150)	
Total other expense, net	**(565,000)**	
Loss from operations before income taxes	(5,152,788)	
Provision for income taxes	—	
Net loss and comprehensive loss	**$ (5,152,788)**	
Net loss per common share, basic and diluted(1)	(0.34)	
Weighted average number of shares outstanding used in computation of net loss per common share, basic and diluted	15,270,957	
Pro forma net loss per common share, basic and diluted(1)		
Pro forma weighted average number of shares outstanding used in computation of net loss per common share, basic and diluted(1)		

(1) Unaudited pro forma net loss per share, basic and diluted, is calculated giving effect to the automatic conversion of the convertible preferred stock into shares of common stock. Unaudited pro forma net loss per share does not include the shares expected to be sold and related proceeds to be received in this offering. Unaudited pro forma net loss per share for the year ended December 31, 2020 and 2021 was calculated using the weighted-average number of shares of common stock outstanding, including the pro forma effect of the automatic conversion of all outstanding

shares of our convertible preferred stock into shares of our common stock, as if the initial public offering had occurred at the beginning of the period or their issuance dates, if later.

	As of December 31, 2021		
	Actual	Pro forma(1)	Pro forma as adjusted(2)
Balance Sheet Data:			
Cash	$	$	$
Working capital(3)			
Total assets			
Convertible preferred stock			
Total stockholders' equity (deficit)			

(1) The pro forma column reflects the conversion of all of the outstanding preferred shares of our redeemable convertible preferred stock into an aggregate of shares of our common stock upon completion of this offering.

(2) The pro forma as adjusted column reflects the pro forma adjustments set forth above and the sale of shares of common stock in this offering at an assumed initial public offering price of $ per share, which is the midpoint of the price range set forth on the cover page of this prospectus, after deducting estimated underwriting fees and commissions and estimated offering expenses payable by us. This pro forma as adjusted information is illustrative only and will depend on the actual initial public offering price and other terms of this offering determined at pricing. Each $1.00 increase or decrease in the assumed initial public offering price of $ per share, which is the midpoint of the price range set forth on the cover page of this prospectus, would increase or decrease the pro forma as adjusted amount of each of cash, working capital, total assets and total stockholders' equity by $ million, assuming that the number of shares offered by us, as set forth on the cover page of this prospectus, remains the same and after deducting the estimated underwriting discounts and commissions. Each increase or decrease of 1.0 million in the number of shares we are offering would increase or decrease the pro forma as adjusted amount of each of cash, working capital, total assets and total stockholders' equity by $ million, assuming no change in the assumed initial public offering price per share and after deducting the estimated underwriting discounts and commissions.

(3) We define working capital as current assets less current liabilities. See our financial statements appearing elsewhere in this prospectus.

Risk factors

Investing in our common stock involves a high degree of risk. You should consider carefully the risks and uncertainties described below, together with all of the other information in this prospectus, including our financial statements and related notes, before deciding whether to purchase shares of our common stock. The risks and uncertainties described below are not the only ones we face. Additional risks and uncertainties that we are unaware of, or that we currently believe are not material, may also become important factors that affect us. If any of the following risks are realized, our business, financial condition, results of operations and prospects could be materially and adversely affected. In that event, the price of our common stock could decline, and you could lose part or all of your investment.

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, 2020 and 2021, we incurred a net loss of $5.2 and $, respectively, and we had an accumulated deficit of $ as of December 31, 2021. We have financed our operations with approximately $183.7 in gross proceeds raised in our private placements of preferred stock through August 2021. 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 product candidates, and only one of our product candidates has completed a Phase 1/2 clinical trial. 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 current good manufacturing practices, or 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 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 gene therapy 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 initiate or 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.

Even if this offering is successful, 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. Furthermore, upon the closing of this offering, we 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, 2021, we had cash of $. We believe that the anticipated net proceeds from this offering, together with our existing cash, will be sufficient to fund our operating expenses and capital requirements through . 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;
- the extent to which we develop, in-license or acquire other product candidates and technologies in our gene therapy 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 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. Additional funds may not be available on a timely basis, on favorable terms, or at all, and such funds, if raised, may not be sufficient to enable us to continue to implement our long-term business strategy. Further, our ability to raise additional capital may be adversely impacted by potential worsening global economic conditions and the recent disruptions to and volatility in the credit and financial markets in the United States and worldwide resulting from the ongoing COVID-19 pandemic, including new variants of the virus, such as the Delta and Omicron variants. If we are unable to raise sufficient additional capital, 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 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 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, LX1004 and any other product candidates in a timely manner.

Each of our product candidates and programs will require additional preclinical and 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 FDA's good laboratory practices, or 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 investigational new drug applications, or INDs, from the U.S. Food and Drug Administration, or 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 the FDA's current Good Clinical Practices, or cGCPs;
- establishment of 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;
- 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 diseases;
- 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 product candidate, and any diseases for such product candidate, that we develop;
- 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, including cGMPs, and complying effectively with other procedures;
- attainment and maintenance of third-party coverage and adequate reimbursement 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 efficacious 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. Only two AAV-based product candidates are currently approved by the FDA, and both of these use a different vector than our current product candidates.

We cannot be certain that our AAVrh10-based gene therapy product candidates or any future product candidates utilizing other vector constructs will successfully complete preclinical studies and 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, intrathecal and intraparenchymal 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 attractive. 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 can predict neither the geographic areas in which we could obtain regulatory approval nor 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 further 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 therapeutic 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. Moreover, there is substantial overlap in those responsible for regulation of existing gene therapy products. For example, in the United States, the FDA has established the Office of Tissues and Advanced Therapies within its Center for Biologics Evaluation and Research, or CBER, to consolidate the review of gene therapy and related products, and the Cellular, Tissue and Gene Therapies Advisory Committee to advise CBER on its review.

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. In addition to FDA oversight and oversight by institutional review boards, or IRBs, certain human clinical trials involving recombinant or synthetic nucleic acid molecules are subject to oversight of institutional biosafety committees, or IBCs, as set forth in the NIH Guidelines. Under the NIH Guidelines, recombinant and synthetic nucleic acids are defined as: (i) molecules that are constructed by joining nucleic acid molecules and that can replicate in a living cell (i.e., recombinant nucleic acids); (ii) nucleic acid molecules that are chemically or by other means synthesized or amplified, including those that are chemically or otherwise modified but can base pair with naturally occurring nucleic acid molecules (i.e., synthetic nucleic acids); or (iii) molecules that result from the replication of those described in (i) or (ii). Specifically, under the NIH Guidelines, supervision of human gene transfer trials includes evaluation and assessment by an IBC, a local institutional committee that reviews and oversees research utilizing recombinant or synthetic nucleic acid molecules at that institution. The IBC assesses the safety of the research and identifies any potential risk to public health or the environment and such review may result in some delay before initiation of a clinical trial. While the NIH Guidelines are not mandatory unless the research in question is being conducted at or sponsored by institutions receiving NIH funding of recombinant or synthetic nucleic acid molecule research, many companies and other institutions not otherwise subject to the NIH Guidelines voluntarily follow them. Although the FDA decides whether individual gene therapy protocols may proceed, the review process and determinations of other reviewing bodies can impede or delay the initiation of a clinical trial, even if the FDA has reviewed the trial and approved its initiation.

The same applies in the European Union. 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 European Union, 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. As a result, the procedures and standards applied to gene therapy products may be applied to any gene therapy product candidate we may develop, but that remains uncertain at this point.

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 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 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 intend to 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 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 and 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, clinical trials of LX1001 and LX1004 have been initiated and conducted by our collaborators, and we have not successfully initiated or 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 very rare diseases, such as Batten disease, where the very small patient population makes it difficult or impossible to conduct two traditional, adequate and well-controlled studies, 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 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;
- obtaining regulatory approval to commence a clinical trial;
- reaching an agreement on acceptable terms with clinical trial sites or prospective contract research organizations, or CROs, the terms of which can be subject to extensive negotiation and may vary significantly among different clinical trial sites;
- obtaining IRB approval for each clinical trial site;
- recruiting suitable patients to participate in a clinical trial in a timely manner;
- having patients complete a clinical trial or return for post-treatment follow-up;
- clinical trial sites, CROs or other third parties deviating from trial protocol or dropping out of a trial;
- failure to perform our planned clinical trials in accordance with the FDA's cGCP requirements, or applicable regulatory guidelines in other countries;
- addressing 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;
- adding a sufficient number of clinical trial sites; or
- manufacturing sufficient quantities of 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 abandon 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;
- 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 Risk Evaluation and Mitigation Strategies, 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 biologics license application, or 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 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 biologic product candidates in the United States until we receive regulatory approval of a BLA from the FDA, and we cannot market it in the European Union until we receive approval for a 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 are promising, 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, LX1001, LX1004 and any 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.

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.

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 large-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 Therapeutics, Inc. and UCSD for our product candidates LX2020, 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.

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. The Phase 1/2 clinical trial of LX1004 for the treatment of neuronal ceroid lipofuscinosis type 2 (CLN2) disease, or CLN2 Batten disease, employed an intraparenchymal route of administration. We are planning to pursue an intracisternal route of administration in our anticipated potentially pivotal Phase 2/3 clinical trial of LX1004 and results from the Phase 1/2 clinical trial may not be indicative of future trials that we conduct using a different route of administration. Furthermore, the clinical trial of LX1004, as well as our other past 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 with any of our product candidates for which we include an open-label clinical trial 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 or publish 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 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 one case of hepatocellular carcinoma, or HCC, in a muscle creatine kinase, or MCK, mouse at 10 months post-treatment. Although a large body of available data 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.

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 central nervous system, or CNS, product candidates are designed to be delivered either via intracisternal or parenchymal administration. While the intracisternal and parenchymal methods of administration have been available for some years, their use for gene therapies is new and no gene therapy is currently approved for these methods 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, while parenchymal administration involves neurosurgery and carries all risks attendant with such a procedure. These risks include allergic reaction to anesthesia, bleeding or swelling in the brain,

seizures, stoke, coma and infection. Other gene therapy product candidates in clinical development utilizing intracisternal or parenchymal delivery could also generate data that could adversely affect the clinical, regulatory or commercial perception of our product candidates.

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 LX1004 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 disease, 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 achieve 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 size and nature of the patient population and competition for patients with other trials. Genetic diseases generally, and especially the rare disease for which some of our product candidates are designed to target, have low incidence and prevalence. 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 there are approximately 8,000 people in the United States who have Friedreich's ataxia and that approximately 70% of these patients will develop the cardiac manifestation of Friedreich's ataxia, 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. 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 and 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 novelty of gene therapy as a modality for treatment of our target indications;
- the availability of competing commercially available therapies and other competing therapeutic 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;
- potential disruptions caused by the ongoing COVID-19 pandemic (including new variants of the virus, such as the Delta and Omicron variants), including difficulties in initiating clinical sites, enrolling and retaining participants, diversion of healthcare resources away from clinical trials, travel or quarantine policies that may be implemented, and other factors;
- 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 expect to rely on CROs and clinical trial sites to ensure the proper and timely conduct of our clinical trials and we will have limited influence over their performance.

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.

We may seek orphan drug designation or rare pediatric drug 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 LX1004 for treatment of CLN2 Batten disease and LX2006 for treatment of Friedreich’s ataxia. 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 law reverses 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 did not further change this interpretation when it clarified that the interpretation codified in FDARA would apply in cases where FDA issued an orphan designation before the enactment of FDARA but where product 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. 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. The FDA may further reevaluate the Orphan Drug Act and its regulations and policies. We do not know if, when, or how the FDA 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.

Fast track, breakthrough therapy, or regenerative medicine advanced therapy designation by 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 may seek fast track, breakthrough therapy or regenerative medicine advanced 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. FDA's fast track, breakthrough therapy, and RMAT designation programs are intended to expedite the development of certain qualifying products 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. 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. While we may seek fast track, breakthrough and/or RMAT designation for some or all of our product candidates, there is no guarantee that we will be successful in 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, or RMAT designation does not ensure that the product candidate will receive marketing approval or that approval will be granted within any particular timeframe. In addition, the FDA may withdraw fast track, breakthrough, or RMAT designation if it believes that the designation is no longer supported by data from our clinical development program. Fast track, breakthrough and/or RMAT designation alone do 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 Friedreich's ataxia and LX1004 for the treatment of neuronal ceroid lipofuscinosis type 2 (CLN2) disease and we may seek such designation for certain other 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 Friedreich's ataxia and LX1004 for the treatment of neuronal ceroid lipofuscinosis type 2 (CLN2) disease and we may seek rare pediatric disease designation for certain of our other 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 U.S. Federal Food, Drug, and Cosmetic Act, or 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, do not meet the eligibility criteria for a priority review voucher, including for the following reasons:

- the rare pediatric disease for which 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 or LX1004 in patients with Friedreich's ataxia or CLN2 disease, respectively, 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.

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 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 irreversible morbidity or mortality. 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 irreversible morbidity or mortality that is reasonably likely to predict an effect on irreversible morbidity or mortality 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 LX2006, LX1001 and LX1004. As a result, we may forego or delay pursuit of opportunities with other product candidates. 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 therapies for patients with both rare and prevalent cardiac and central nervous system diseases by establishing focused selection criteria 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 harmful 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 may 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 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 may 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.

Our business and operations may be adversely affected by the evolving and ongoing COVID-19 global pandemic.

Our business and operations may be adversely affected by the effects of the evolving COVID-19 virus, which was declared by the World Health Organization as a global pandemic, including the current global resurgences as a result of the Delta and Omicron variants and other future resurgences. The ongoing COVID-19 pandemic has resulted in travel and other restrictions in order to reduce the spread of the disease, including public health directives and orders in the United States and the European Union that, among other things and for various periods of time, directed individuals to shelter at their places of residence, directed businesses and governmental agencies to cease non-essential operations at physical locations, prohibited certain non-essential gatherings and events and ordered cessation of non-essential travel. Future remote work policies and similar government orders or other restrictions on the conduct of business operations related to the COVID-19 pandemic may negatively impact productivity; disrupt our ongoing research and development activities and our clinical programs and timelines; and cause disruptions to our supply chain, to the administrative functions of clinical trial sites and to the operations of our other partners, the magnitude of which will depend, in part, on the length and severity of the restrictions and other limitations on our ability to conduct our business in the ordinary course. In the event that government authorities were to enhance current restrictions, our employees who currently are not telecommuting may no longer be able to access our facilities, including our laboratories, and our operations may be further limited or curtailed. Further, such orders also may impact the availability or cost of materials, which would disrupt our supply chain and manufacturing efforts and could affect our ability to conduct ongoing and planned clinical trials and preparatory activities. We may also face difficulties in obtaining access to manufacturing slots for our product candidates. For example, since the beginning of the COVID-19 pandemic, three vaccines for COVID-19 have received Emergency Use Authorization by the FDA, and two of those later received marketing approval. Additional vaccines may be authorized or approved in the future. The resultant demand for vaccines and potential for manufacturing facilities and materials to be commandeered under the Defense Production Act of 1950, or equivalent foreign legislation, may make it more difficult to obtain materials or manufacturing slots for the products needed for our clinical trials, which could lead to delays in these trials.

We have experienced delays in patient enrollment and certain follow up visits in the Phase 1/2 clinical trial of LX1001, and we may experience additional disruptions related to the COVID-19 pandemic in the future that could severely impact our ongoing and planned clinical trials, including:

- delays, difficulties or a suspension in clinical site initiation, including difficulties in recruiting clinical site investigators and clinical site staff;
- delays, difficulties or interruptions in shipping and delivering in a timely manner supplies, samples or products required for our clinical trials due to the impact of the ongoing COVID-19 pandemic on the United States Postal Service, FedEx, United Parcel Service and/or other commercial shipping organizations;
- delays, difficulties or interruptions in obtaining the raw materials and other resources needed for our operations, including due to government-led diversion, reprioritization or appropriation of such resources;
- interruptions or delays in patient enrollment or patient visits on trial due to quarantines, perceived risks of in person visits or limited facility and healthcare professional availability;
- delays or interruptions in third-party or collaborator services, including due to government-led diversion, reprioritization or appropriation of such services;
- interruptions in our ability to manufacture and deliver drug supply for trials;
- diversion of healthcare resources away from the conduct of clinical trials, including the diversion of hospitals serving as our clinical trial sites and hospital staff supporting the conduct of our clinical trials;

- changes in local regulations as part of a response to the COVID-19 outbreak that may require us to change the ways in which our clinical trials are conducted, which may result in unexpected costs, or to discontinue the clinical trials altogether;
- interruption of key clinical trial activities, such as clinical trial site monitoring, and the ability or willingness of subjects to travel to trial sites due to limitations on travel imposed or recommended by federal or state governments, employers and others;
- limitations in employee resources that would otherwise be focused on the conduct of our clinical trials, including because of sickness of employees or their families or the desire of employees to avoid contact with large groups of people;
- interruption or delays in the operations of the FDA, EMA or other regulatory authorities, which may impact review and approval timelines;
- delays in necessary interactions with local regulators, ethics committees and other important agencies and contractors due to limitations in employee resources or forced furlough of government employees; and
- refusal of the FDA to accept data from clinical trials in these affected geographies.

The spread of COVID-19, including new variants of the virus such as the Delta and Omicron variants, which has caused a broad impact globally, may materially affect us economically. While the overall economic impact brought by, and the duration of, COVID-19 may be difficult to assess or predict, the ongoing pandemic could result in further disruption of global financial markets, reducing our ability to access capital, which could in the future negatively affect our liquidity. In addition, a recession or market correction resulting from the spread of COVID-19 could materially affect our business and the value of our common stock.

The global COVID-19 pandemic continues to rapidly evolve. The extent to which the COVID-19 pandemic impacts our business and operations, including our clinical development and regulatory efforts, will depend on future developments that are highly uncertain and cannot be predicted with confidence at the time of this prospectus, such as the duration of the outbreak, the duration and effect of business disruptions, the occurrence of global resurgences as a result of the Delta and Omicron variants and other future resurgence, and the short-term effects and ultimate effectiveness of the travel restrictions, quarantines, social distancing requirements and business closures in the United States and other countries to contain and treat the disease. Accordingly, we do not yet know the full extent of potential delays or impacts on our business, our clinical and regulatory activities, healthcare systems or the global economy as a whole. However, these impacts could adversely affect our business, financial condition, results of operations and growth prospects.

In addition, to the extent the ongoing COVID-19 pandemic adversely affects our business and results of operations, it may also have the effect of heightening many of the other risks and uncertainties described in this “Risk Factors” section.

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 record keeping) 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 or 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 contract development and manufacturing organizations 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 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 contract manufacturing organizations, or 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 were to fail to comply with the requirements of the FDA, EMA or other regulatory authority, it could result in sanctions being 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.

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 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 all of 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 are likely to 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 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:

- the efficacy, safety and potential advantages compared to alternative treatments;
- our ability to offer our products for sale at competitive prices;
- the 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 black box 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 potential therapeutic products 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 2021, sponsors of other clinical trials involving gene therapies have announced imposition of clinical holds by 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 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 candidates which require that the expression of a therapeutic transgene be tightly regulated, such as LX1020, we may inadvertently cause overexpression, which could lead to numerous issues, including safety and toxicity concerns. Furthermore, these regulatory gene replacement therapy candidates require the insertion of microRNA, or 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 orphan 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 appropriate, and the methodology is forward-looking and 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 with caution. 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 and CNS 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 Novartis International AG, or Novartis, PTC Therapeutics, Inc., Aavanti Bio, Inc., Lacerta Therapeutics, Inc. and those being developed in collaborations between Voyager Therapeutics, Inc., and Neurocrine Biosciences, Inc. and between Takeda Pharmaceutical Company Limited and StrideBio, Inc. Among other treatment modalities for Friedreich's ataxia, we are aware that Larimar Therapeutics, Inc. is developing a clinical stage product candidate, CTI-1601, and that Reata Pharmaceuticals, Inc. is developing late-clinical-stage candidate omaveloxolone. 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 toxic variant while expressing the protective variant and Novartis has a gene therapy 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 aducanumab (Aduhelm), which was recently approved under accelerated approval and will require confirmatory data to verify clinical benefit. Finally, we are aware that Voyager Therapeutics, Inc. and Taysha Gene Therapies, Inc. are pursuing Alzheimer's disease treatments and have early-stage discovery efforts ongoing based on vectorized antibodies and tau-specific miRNA shuttles, respectively. With respect to LX1004, the FDA approved BioMarin Pharmaceutical, Inc.'s treatment for CLN2 Batten disease, cerliponase alfa (Brineura) in 2017. Brineura is an enzyme replacement therapy which requires chronic twice monthly infusions directly into the cerebrospinal fluid, or CSF, via intraventricular injections. In addition to Brineura, REGENXBIO Inc. is developing two preclinical gene therapy candidates to treat the CNS and ocular manifestations of CLN2 Batten disease. With respect to LX2020, Tenaya Therapeutics Inc. is developing a preclinical AAV-based gene therapy candidate designed to deliver a functional *PKP2* gene to patients with arrhythmogenic right ventricular cardiomyopathy, or ARVC.

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 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 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 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 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 their 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 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 LX1004, 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 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.

The principal decisions about reimbursement for new medicines are typically made by the Centers for Medicare & Medicaid Services, or CMS, an agency within the U.S. Department of Health and Human Services, or 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.

Reimbursement by a third-party payor may depend upon a number of factors, including the third-party payor's determination that a procedure is 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, LX1004, 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 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 for many of our preclinical and clinical research and development programs, including for discovering, developing and conducting IND-enabling studies for portions of our near-term future pipeline. Failure or delay of Cornell University to fulfill all or part of its obligations to us under the agreement, a breakdown in collaboration between the parties or a complete or partial loss of this relationship would materially harm our business.

Our company was founded with a collaboration between us, Cornell University, and Ronald G. Crystal, M.D., Professor and Chairman of Weill Cornell Medicine's Department of Genetic Medicine. 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. In June 2019, Cornell University completed the first clinical trial for LX1004. As part of our second license agreement, Cornell assigned the IND for LX1004 to us. As part of our first license agreement, 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. Our collaboration with Cornell University provides us with the opportunity to expand our pipeline into several additional cardiac and CNS disease targets. In February 2021, we further expanded our collaboration and entered into a Research Collaboration Agreement with Cornell University to enable discovery and preclinical research aimed at expanding our cardiovascular pipeline. If Cornell University delays or fails to perform its obligations under this collaboration agreement, disagrees with our interpretation of the terms of the collaboration or our discovery plan or terminates any of our existing license and collaboration agreements, our pipeline of product candidates would be significantly adversely affected and our prospects will 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 have engaged CROs to 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. Further, the performance of our CROs may also be interrupted by the ongoing COVID-19 pandemic, including due to travel or quarantine policies, heightened exposure of CRO staff who are healthcare providers to COVID-19 or prioritization of resources toward the pandemic.

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, ClinicalTrials.gov, within specified timeframes. 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, 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. 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.

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, LX1004, 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;
- 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, LX1001, LX1004 and other programs, their respective components, formulations, therapies, methods used to manufacture them and methods treatment. We currently do not have any patents or patent applications covering our LX1004 or LX1001 product candidates.

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.

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 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 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 Cornell University and Adverum Biotechnologies, Inc. related to LX2006, and we in-license know-how from Cornell University related to our LX1001 and LX1004 product candidates for the treatment of Alzheimer's disease. 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, 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. 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 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.

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 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 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 related to LX2006 and LX1001. 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;
- 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 do not currently own or license any U.S. composition of matter patents or patent applications covering our LX1001 and LX1004 product candidates and 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. We currently in-license two issued U.S. patents that relates to our LX2006 product candidate. However, we do not own or in-license any composition of matter patents or patent applications in the United States or any other jurisdiction with respect to our LX1001 and LX1004 product candidates. 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 Biotechnologies Inc., 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 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 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 may be subject to claims that our employees, consultants, or independent contractors have wrongfully used or disclosed confidential information 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 we are successful, litigation could result in substantial cost and be a distraction to our management and other employees. 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 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) that should be listed as inventor(s) or include individual(s) that 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;
- 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 enjoy only 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 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 allow 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 relationships with customers, healthcare providers, including physicians, and third-party payors are 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.

Healthcare providers, including physicians, and third-party payors in the United States and elsewhere will play a primary role in the recommendation and prescription of any product candidates for which we obtain marketing approval. Our current and future arrangements with healthcare professionals, principal investigators, consultants, customers and third-party payors subject us to various federal and state fraud and abuse laws and other healthcare laws, including, without limitation, the U.S. federal Anti-Kickback Statute, the federal civil and criminal false claims laws and the law commonly referred to as the Physician Payments Sunshine Act and regulations promulgated under such laws. These laws will impact, among other things, our clinical research, proposed sales, marketing and educational programs, and other interactions with healthcare professionals. In addition, we may be subject to patient privacy laws by both the federal government and the states in which we conduct or may conduct our business. The laws that will affect our operations include, but are not limited to:

- the U.S. federal Anti-Kickback Statute, which prohibits, among other things, individuals or entities from knowingly and willfully soliciting, receiving, offering or paying any remuneration (including any kickback,

bribe or rebate), directly or indirectly, overtly or covertly, in cash or in kind in return for, or to induce, either the referral of an individual, or the purchase, lease, order or arrangement for or recommendation of the purchase, lease, order or arrangement for any good, facility, item or service for which payment may be made, in whole or in part, under a federal healthcare program, such as the Medicare and Medicaid programs. The term "remuneration" has been broadly interpreted to include anything of value. Although there are a number of statutory exceptions and regulatory safe harbors protecting some common activities from prosecution, the exceptions and safe harbors are drawn narrowly. Practices that involve remuneration that may be alleged to be intended to induce prescribing, purchases or recommendations may be subject to scrutiny if they do not qualify for an exception or safe harbor. A person does not need to have actual knowledge of this statute or specific intent to violate it in order to have committed a violation. In addition, the ACA, signed into law in 2010, provides that the government may assert that 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 federal False Claims Act;

- the federal civil and criminal false claims laws, including, without limitation, the federal False Claims Act, which can be enforced by private citizens through civil whistleblower or *qui tam* actions, and the federal civil monetary penalty law which prohibit, among other things, individuals or entities from knowingly presenting, or causing to be presented, claims for payment or approval from the federal government, including Medicare, Medicaid and other government payors, that are false or fraudulent or knowingly making, using or causing to be made or used a false record or statement material to a false or fraudulent claim or to avoid, decrease or conceal an obligation to pay money to the federal government. A claim includes "any request or demand" for money or property presented to the United States federal government. Several pharmaceutical and other healthcare companies have been prosecuted under these laws for allegedly providing free product to customers with the expectation that the customers would bill federal programs for the product. Other companies have been prosecuted for causing false claims to be submitted because of the companies' marketing of products for unapproved, and thus non-reimbursable, uses;

- the federal Health Insurance Portability and Accountability Act of 1996, or HIPAA, which created additional federal criminal statutes which prohibit, among other things, a person from knowingly and willfully executing, or attempting to execute, a scheme to defraud any healthcare benefit program, including private third-party payors and knowingly and willfully falsifying, concealing or covering up a material fact or making any materially false, fictitious or fraudulent statement in connection with the delivery of or payment for healthcare benefits, items or services. Similar to the U.S. federal Anti-Kickback Statute, 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;

- HIPAA, as amended by the Health Information Technology for Economic and Clinical Health Act of 2009, or HITECH, and their implementing regulations, which imposes certain requirements relating to the privacy, security and transmission of individually identifiable health information on health plans, healthcare clearinghouses and certain healthcare providers, known as "covered entities", and their respective HIPAA "business associates", which are independent contractors that perform certain services for or on behalf of covered entities or other business associates involving the use or disclosure of individually identifiable health information. 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 HIPAA and seek attorneys' fees and costs associated with pursuing federal civil actions;

- the federal transparency laws, including the U.S. federal Physician Payments Sunshine Act, which requires certain manufacturers of drugs, medical devices, biologicals and medical supplies for which payment is available under Medicare, Medicaid or the State Children's Health Insurance Program, with specific exceptions, to report annually to CMS information related to: (i) payments or other "transfers of value" made

to physicians (defined to include doctors, dentists, optometrists, podiatrists and chiropractors) and teaching hospitals, and (ii) ownership and investment interests held by physicians and their immediate family members. Effective January 1, 2022, these reporting obligations extended to include payments and other transfers of value made, as well as ownership and investment interests held, during the previous year to certain non-physician providers, including physician assistants, nurse practitioners clinical nurse specialists, anesthesiologist assistants, certified registered nurse anesthetists and certified nurse midwives; and

- state and foreign laws and regulations that are analogous to each of the above federal laws; state laws that require manufacturers to report information related to payments and other transfers of value to physicians and other healthcare providers, marketing expenditures or drug pricing; state laws that require pharmaceutical companies to comply with the pharmaceutical industry's voluntary compliance guidelines and the relevant compliance guidance promulgated by the federal government, or that otherwise restrict payments that may be made to healthcare providers; state and local laws that require the registration of pharmaceutical sales representatives; and state and foreign laws that govern the privacy and security of health information in some circumstances, many of which differ from each other in significant ways and often are not preempted by HIPAA, thus complicating compliance efforts.

Because of the breadth of these laws and the narrowness of the statutory exceptions and regulatory safe harbors available, it is possible that some of our business activities could be subject to challenge under one or more of such laws. It is possible that governmental authorities will conclude that our 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 our 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.

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, and we do not have experience in obtaining regulatory approval 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, 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.

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 example, in March 2010, the ACA was enacted, which substantially changed the way healthcare is financed by both governmental and private insurers. Among the provisions of the ACA, those of greatest importance to the pharmaceutical and biotechnology industries include the following:

- an annual, non-deductible fee payable by any entity that manufactures or imports certain branded prescription drugs and biologic agents (other than those designated as orphan drugs), which is apportioned among these entities according to their market share in certain government healthcare programs;
- a new methodology by which rebates owed by manufacturers under the Medicaid Drug Rebate Program are calculated for drugs that are inhaled, infused, instilled, implanted or injected;
- expansion of eligibility criteria for Medicaid programs by, among other things, allowing states to offer Medicaid coverage to certain individuals with income at or below 133% of the federal poverty level, thereby potentially increasing a manufacturer's Medicaid rebate liability;
- a licensure framework for follow on biologic products;
- a Patient-Centered Outcomes Research Institute to oversee, identify priorities in, and conduct comparative clinical effectiveness research, along with funding for such research; and
- establishment of a Center for Medicare & Medicaid Innovation at CMS to test innovative payment and service delivery models to lower Medicare and Medicaid spending, potentially including prescription drug spending.

Since its enactment, there have been judicial, Congressional and executive branch challenges to certain aspects of the ACA. While Congress has not passed comprehensive repeal legislation, it has enacted laws that modify certain provisions of the ACA such as removing penalties, starting January 1, 2019, for not complying with the ACA's individual mandate to carry health insurance, delaying the implementation of certain ACA-mandated fees, and increasing the point-of-sale discount that is owed by pharmaceutical manufacturers who participate in Medicare Part D. On June 17, 2021 the U.S. Supreme Court dismissed a challenge on procedural grounds that argued the ACA is unconstitutional in its entirety because the "individual mandate" was repealed by Congress. Thus, the ACA will remain in effect in its current form. Further, prior to the U.S. Supreme Court ruling, on January 28, 2021, President Biden issued an executive order that initiated a special enrollment period for purposes of obtaining health insurance coverage through the ACA marketplace, which began on February 15, 2021 and remained open until August 15, 2021. The executive order also instructed certain governmental agencies to review and reconsider their existing policies and rules that limit access to healthcare, including among others, reexamining Medicaid demonstration projects and waiver programs that include work requirements, and policies that create unnecessary barriers to obtaining access to health insurance coverage through Medicaid or the ACA. It is possible that the ACA will be subject to judicial or Congressional challenges in the future. It is unclear how any such challenges and the health reform measures of the Biden administration will impact the ACA and our business.

In addition, other legislative changes have been proposed and adopted in the United States since the ACA was enacted. In August 2011, the Budget Control Act of 2011, among other things, led to aggregate reductions of

Medicare payments to providers of 2% per fiscal year. These reductions went into effect in April 2013 and, due to subsequent legislative amendments to the statute will remain in effect through 2031 unless additional action is taken by Congress. However, pursuant to COVID-19 relief legislation, these Medicare sequester reductions have been suspended from May 1, 2020 through December 31, 2021 due to the COVID-19 pandemic. In January 2013, the American Taxpayer Relief Act of 2012 was signed into law, which, among other things, further reduced Medicare payments to several types of providers, including hospitals, imaging centers and cancer treatment centers, and increased the statute of limitations period for the government to recover overpayments to providers from three to five years. On July 9, 2021, President Biden issued an executive order directing the FDA to, among other things, continue to clarify and improve the approval framework for generic drugs and biosimilars, including the standards for interchangeability of biologics, facilitate the development and approval of biosimilar and interchangeable products, clarify existing requirements and procedures related to the review and submission of BLAs, and identify and address any efforts to impede generic drug and biosimilar competition. Additionally, on March 11, 2021, President Biden signed the American Rescue Plan Act of 2021 into law, which eliminates the statutory Medicaid drug rebate cap, currently set at 100% of a drug's average manufacturer price, for single source and innovator multiple source drugs, beginning January 1, 2024. In addition, Congress is considering additional health reform measures as part of the budget reconciliation process. It is also possible that additional governmental action is taken in response to the COVID-19 pandemic.

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. For example, July 2021, the Biden administration released an executive order, "Promoting Competition in the American Economy," with multiple provisions aimed at prescription drugs. This executive order directs the FDA to, among other things, continue to clarify and improve the approval framework for biosimilars, including the standards for interchangeability of biologics, facilitate the development and approval of biosimilar and interchangeable products, clarify existing requirements and procedures related to the review and submission of BLAs, and identify and address any efforts to impede generic drug and biosimilar competition. In response to President Biden's executive order, on September 9, 2021, HHS released a Comprehensive Plan for Addressing High Drug Prices that outlines principles for drug pricing reform and sets out a variety of potential legislative policies that Congress could pursue as well as potential administrative actions HHS can take to advance these principles. No legislation or administrative actions have been finalized to implement these principles. 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 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.

Additionally, as of May 26, 2021, the FDA noted it is continuing to ensure timely reviews of applications for medical products during the ongoing COVID-19 pandemic in line with its user fee performance goals and conducting mission critical domestic and foreign inspections to ensure compliance of manufacturing facilities with FDA quality standards. However, the FDA may not be able to continue its current pace and approval timelines could be extended, including where a pre-approval inspection or an inspection of clinical sites is

required and due to the ongoing COVID-19 pandemic and travel restrictions FDA is unable to complete such required inspections during the review period.

Since March 2020 when foreign and domestic inspections of facilities were largely placed on hold, the FDA has been working to resume routine surveillance, bioresearch monitoring and pre-approval inspections on a prioritized basis. Since April 2021, the FDA has conducted limited inspections and employed remote interactive evaluations, using risk management methods, to meet user fee commitments and goal dates. Ongoing travel restrictions and other uncertainties continue to impact oversight operations both domestic and abroad and it is unclear when standard operational levels will resume. The FDA is continuing to complete mission-critical work, prioritize other higher-tiered inspectional needs (e.g., for-cause inspections), and carry out surveillance inspections using risk-based approaches for evaluating public health. Should the FDA determine that an inspection is necessary for approval and an inspection cannot be completed during the review cycle due to restrictions on travel, and the FDA does not determine a remote interactive evaluation to be adequate, the agency has stated that it generally intends to issue, depending on the circumstances, a complete response letter or defer action on the application until an inspection can be completed. During the COVID-19 public health emergency, a number of companies announced receipt of complete response letters due to the FDA's inability to complete required inspections for their applications. Regulatory authorities outside the United States may adopt similar restrictions or other policy measures in response to the COVID-19 pandemic and may experience delays in their regulatory activities. 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 business. Further, future shutdowns of other government agencies, such as the SEC, may also impact our business through review of our public filings and our ability to access the public markets.

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, 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 LX1004, 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 LX1004, or any of our other product candidates, if approved, we may be forced to delay the potential commercialization of LX2006, LX1001 or LX1004 or any of our other product candidates or reduce the scope of our sales or marketing activities for LX2006, LX1001 or LX1004 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 LX1004 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 LX1004 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 or LX1004 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;
- 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, 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.

We have no prior experience in these areas. 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.

Risks related to employee matters and managing our growth

Our future success depends on our ability to 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, Jay A. Barth, M.D., our Executive Vice President and Chief Medical Officer and Paul McCormac, Ph.D., our Chief Technical 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 scientific 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 could impede the achievement of our development and commercialization objectives and seriously harm our ability to successfully implement our business strategy. Furthermore, 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 may be unable to hire, train, retain or motivate these 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, 2021, we had 24 full-time employees and no part-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 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. In connection with this offering, we intend to adopt a code of business conduct and ethics; however, even with such a code of conduct in place, 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.

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

Despite the implementation of security measures, our internal computer systems, as well as those of third 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 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 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 data breach notification laws, state health information privacy laws and federal and state consumer protection laws and regulations, including Section 5 of the Federal Trade Commission Act, that govern the collection, use, disclosure and protection of health-related and other personal information. In addition, 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, as amended by the Health Information Technology for Economic and Clinical Health Act, and the regulations promulgated thereunder. 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 Europe, the General Data Protection Regulation, or GDPR, took effect in May 2018. The GDPR governs the collection, use, disclosure, transfer or other processing of personal data of individuals within the European Economic Area, or EEA, including clinical trial data. Among other things, the GDPR imposes requirements regarding the security of personal data and notification of data breaches to the competent national data processing authorities, requires having lawful bases on which personal data can be processed and requires changes to

informed consent practices, as well as more detailed notices for clinical trial subjects and investigators. In addition, the GDPR increases the scrutiny of transfers of personal data from the EEA to the United States and other jurisdictions that the European Commission does not recognize as having "adequate" data protection laws; in July 2020, the Court of Justice of the European Union limited how organizations could lawfully transfer personal data from the EEA to the United States by invalidating the EU-U.S. Privacy Shield and imposing further restrictions on the use of standard contractual clauses, which could increase our costs and our ability to efficiently process personal data from the EEA. The GDPR imposes substantial fines for breaches and violations (up to the greater of €20 million 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.

Relatedly, following the United Kingdom's withdrawal from the EEA and the European Union and the expiration of the Transition Period, companies must comply with both the GDPR and the legislation similar to the GDPR as incorporated into UK national law, which provides for significant fines of up to the greater of £17.5 million or 4% of global turnover and exposes companies to two parallel regimes with potentially divergent enforcement actions for certain violations. On January 1, 2021, the United Kingdom became a third country for purposes of the GDPR. The relationship between the United Kingdom and the European Union in relation to certain aspects of data protection law remains unclear, for example with respect to how data can lawfully be transferred between each jurisdiction, which exposes us to further compliance risk. Pursuant to the EU-UK Trade and Cooperation Agreement of December 24, 2020, transfers of personal data from the European Union to the United Kingdom may continue to take place without a need for additional safeguards during a further transition period, which expires on the earlier of (i) the date on which an adequacy decision with respect to the United Kingdom is adopted by the European Commission; or (ii) the expiry of four months, which shall be extended by a further two months unless either the European Union or the United Kingdom objects. On February 19, 2021 the European Commission published its draft decision finding the United Kingdom to be adequate under the GDPR, though it remains unclear whether the European Commission will formally adopt an adequacy decision with respect to the United Kingdom. In the absence of such decision, after the expiry of the additional transition period we may need to put in place additional safeguards for transfers of personal data from the European Union to the United Kingdom, such as standard contractual clauses approved by the European Commission.

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, recently passed in California and will impose 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. It also created a new California data protection agency authorized to issue substantive regulations and could result in increased privacy and information security enforcement. The majority of the provisions will

go into effect on January 1, 2023, and additional compliance investment and potential business process changes may be required. Although the CCPA currently exempts certain health-related information, including clinical trial data, the CCPA and the CPRA may increase our compliance costs and potential liability if we expand our operations into California. Similar laws have been proposed in other states and at the federal level and, if passed, such laws may have potentially conflicting requirements that would make compliance challenging.

With the GDPR, CCPA, CPRA 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 are currently in the process of developing and updating our policies and procedures in accordance with requirements under applicable data privacy and protection laws and regulations. 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.

Risks related to this offering, ownership of our common stock and our status as a public company

An active trading market for our common stock may not develop and you may not be able to resell your shares of our common stock at or above the initial offering price, if at all.

Prior to this offering, there has been no public market for our common stock. The initial public offering price for our common stock was determined through negotiations with the underwriters and may not be indicative of the price at which our common stock will trade after the closing of this offering. An active trading market for our shares may never develop or be sustained following this offering. If an active market for our common stock does not develop or is not sustained, it may be difficult for you to sell shares you purchased in this offering at an attractive price or at all.

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

Our stock price may be volatile. 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 market price for our common stock may be influenced by many factors, including:

- the reporting of unfavorable preclinical and clinical results;
- the commencement, enrollment or results of our clinical trials of LX2006, LX1001 or LX1004 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 or LX1004 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 LX1004 or any other product candidate;
- 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 market 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;
- general political and economic conditions; and
- other events or factors, many of which are beyond our control.

The stock market in general, and the market for biotechnology companies in particular, have experienced extreme price and volume fluctuations that have often been unrelated or disproportionate to the operating performance of these companies, including very recently in connection with the ongoing COVID-19 pandemic, which has resulted in decreased stock prices for many companies notwithstanding the lack of a fundamental change in their underlying business models or prospects. Broad market and industry factors, including potentially worsening economic conditions and other adverse effects or developments relating to the ongoing COVID-19 pandemic, may negatively affect the market price of our common stock, regardless of our actual operating performance. The realization of any of the above risks or any of a broad range of other risks, including those described in this section, could have a significant and material adverse impact on the market price of our common stock.

In addition, in the past, stockholders have initiated class action lawsuits against pharmaceutical and biotechnology companies following periods of volatility in the market prices of these companies' stock. Such litigation, if instituted against us, could cause us to incur substantial costs and divert management's attention and resources from our business.

A significant portion of our total outstanding shares are restricted from immediate resale but may be sold into the market in the near future. This could cause the market 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 market price of our common stock. After this offering, we will have shares of common stock outstanding based on the number of shares outstanding as of December 31, 2021. This includes the shares that we are selling in this offering, which may be resold in the public market immediately. Following the consummation of this offering, approximately % of our outstanding shares will be subject to a 180-day lock-up period provided under lock-up agreements executed in connection with this offering described in "Underwriting" and restricted from immediate resale under the federal securities laws as described in "Shares Eligible for Future Sale." 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 on behalf of the underwriters. We also intend to register shares of common stock that we may issue under our equity compensation plans. Once we register these shares, they can be freely sold in the public market upon issuance, subject to the lock-up agreements. As restrictions on resale end, the market 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 you purchase shares of common stock in this offering, you will suffer immediate dilution of your investment.

The initial public offering price of our common stock will be substantially higher than the pro forma as adjusted net tangible book value per share of our common stock. Therefore, if you purchase shares of our common stock in this offering, you will pay a price per share that substantially exceeds our pro forma as adjusted net tangible book value per share after this offering. To the extent outstanding options are exercised, you will incur further dilution. Based on an assumed initial public offering price of $ per share, which is the midpoint of the estimated price range set forth on the cover page of this prospectus, you will experience immediate dilution of $ per share, representing the difference between our pro forma as adjusted net tangible book value per share, after giving effect to this offering, and the assumed initial public offering price.

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 will be 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 market price of our common stock. In the event we do 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.

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.

Upon the closing of this offering, based on the number of common stock outstanding as of December 31, 2021, our executive officers, directors and stockholders who owned more than 5% of our outstanding common stock

before this offering and their respective affiliates will, in the aggregate, hold shares representing approximately % of our outstanding common stock. As a result, if these stockholders choose to act together, they would be able to control or 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 control or 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. Any of these actions could adversely affect the market price of our common stock.

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 market 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.

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 Jumpstart Our Business Startups Act of 2012, or 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 this offering, or, if earlier, (i) the last day of the fiscal year in which we have total annual gross revenue of at least $1.07 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 30th, 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 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 share 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.

We will have broad discretion in the use of proceeds from this offering and may invest or spend the proceeds in ways with which you do not agree and in ways that may not increase the value of your investment.

We will have broad discretion over the use of proceeds from this offering. You may not agree with our decisions, and our use of the proceeds may not yield any return on your investment. We expect to use the net proceeds to us from this offering, together with our existing cash, to advance the clinical development of LX2006, LX1001, LX1004 and LX2020, to fund the development of our other product candidates and discovery programs, and for working capital and other general corporate purposes. See "Use of Proceeds." In addition, we may use a portion of the proceeds from this offering to pursue our strategy to in-license or develop additional product candidates. Our failure to apply the net proceeds from this offering effectively could compromise our ability to pursue our growth strategy and we might not be able to yield a significant return, if any, on our investment of these net proceeds. You will not have the opportunity to influence our decisions on how to use our net proceeds from this offering.

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, 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 Delaware General Corporation Law 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 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 that will become effective upon the closing of this offering 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 market 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 Delaware General Corporation Law, or 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.

We could be subject to securities class action litigation.

In the past, securities class action litigation has often been brought against a company following a decline in the market price of its securities. This risk is especially relevant for us because biopharmaceutical companies have experienced significant stock price volatility in recent years. If we face such litigation, it could result in substantial costs and a diversion of management's attention and resources, which could harm our business.

General risks

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 U.S. Foreign Corrupt Practices Act, or 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 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.

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

After the closing of this offering, we will be 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, 2023, 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. Prior to this offering, 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 market 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 other regulatory authorities.

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, including proposals made by the Biden administration (such as increasing the U.S. corporate income tax rate), 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. For example, the Tax Cuts and Jobs Act of 2017, or the Tax Act, enacted many significant changes in U.S. federal tax laws. Future guidance from the Internal Revenue Service, or the IRS, and other tax authorities with respect to the Tax Act may adversely affect us, and certain aspects of the Tax Act could be interpreted differently, changed, repealed, or modified in future legislation, possibly with retroactive effect. One such legislation was the Coronavirus Aid, Relief, and Economic Security Act, or the CARES Act, which modified certain provisions of the Tax Act. In addition, it is uncertain if and to what extent various states will conform to 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 under the Tax Act, as amended by the CARES Act or future U.S. federal tax laws, 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.

We will 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 will 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.

Upon completion of this offering, we will become 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.

Special note regarding forward-looking statements

This prospectus contains forward-looking statements that involve substantial risks and uncertainties. The forward-looking statements are contained principally in the sections titled "Prospectus Summary," "Risk Factors," "Management's Discussion and Analysis of Financial Condition and Results of Operations," "Business" and elsewhere in this prospectus. 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. These statements speak only as of the date of this prospectus and involve known and unknown risks, uncertainties and other important factors that may cause our actual results, performance or achievements to be materially different from any future results, performance or achievements expressed or implied by the forward-looking statements. We have based these forward-looking statements largely on our current expectations and projections about future events and financial trends that we believe may affect our business, financial condition and results of operations. These forward-looking statements include, without limitation, statements about the following:

- 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 IND submissions, initiation of planned clinical trials and timing of expected clinical results for LX2006, LX1001, LX1004 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, LX1004 and any other product candidates for any indication;
- the outbreak of the novel strain of coronavirus disease, COVID-19, including new variants of the virus, such as the Delta and Omicron variants, which could adversely impact our business, including our preclinical studies and clinical trials;
- 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, LX1004 or any other product candidate;
- our ability to successfully commercialize our product candidates;
- 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;
- 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;
- our ability to protect and enforce our intellectual property position for our product candidates, and the scope of such protection;
- our financial performance;
- our expected use of proceeds from this offering;
- 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 JOBS Act.

The foregoing list of risks is not exhaustive. Other sections of this prospectus may include additional factors that could harm our business and financial performance. Moreover, we operate in an evolving environment. New risk factors and uncertainties may emerge from time to time, and it is not possible for management to predict all risk factors and uncertainties. As a result of these factors, we cannot assure you that the forward-looking statements in this prospectus will prove to be accurate. 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, changed circumstances or otherwise. You should, however, review the factors and risks and other information we describe in the reports we will file from time to time with the SEC after the date of 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. You should refer to the “Risk Factors” section of this prospectus for a discussion of important factors that may cause our actual results to differ materially from those expressed or implied by our forward-looking statements.

You should read this prospectus and the documents that we reference in this prospectus and have filed as exhibits to the registration statement, of which this prospectus is a part, completely and with the understanding that our actual future results may be materially different from what we expect. We qualify all of our forward-looking statements by these cautionary statements.

Market and industry data

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 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.

Use of proceeds

We estimate that the net proceeds from our issuance and sale of shares of our common stock in this offering will be approximately $ million (or approximately $ million if the underwriters exercise in full their option to purchase up to additional shares), assuming an initial public offering price of $ per share, which is the midpoint of the price range set forth on the cover page of this prospectus, and after deducting the estimated underwriting discounts and commissions and estimated offering expenses payable by us.

Each $1.00 increase (decrease) in the assumed initial public offering price of $ per share would increase (decrease) the net proceeds to us from this offering by approximately $ million, assuming the number of shares offered by us, as set forth on the cover page of this prospectus, remains the same and after deducting the estimated underwriting discounts and commissions and estimated offering expenses payable by us. Each increase (decrease) of 1.0 million in the number of shares we are offering would increase (decrease) the net proceeds to us from this offering, after deducting the estimated underwriting discounts and commissions and estimated offering expenses payable by us, by approximately $ million, assuming the assumed initial public offering price stays the same. We do not expect that a change in the offering price or the number of shares by these amounts would have a material effect on our intended uses of the net proceeds from this offering, although it may impact the amount of time prior to which we may need to seek additional capital.

As of December 31, 2021, we had a cash balance of $ million. We intend to use the net proceeds from this offering, together with our existing cash, as follows:

- approximately $ million to fund our ongoing and planned clinical development of LX2006 for the treatment of FA cardiomyopathy, including ;
- approximately $ million to fund our ongoing and planned clinical development of LX1001 for the treatment of Alzheimer's disease in *APOE4* homozygous patients, including ;
- approximately $ million to fund our development of LX1004 for the treatment of CLN2 Batten disease, including ;
- approximately $ million to fund the continued development of our other programs and cardiac discovery efforts, including ; and
- the remainder for working capital and other general corporate purposes.

Based on our current operational plans and assumptions, we expect our cash, together with the net proceeds from this offering, will be sufficient to fund our operations through . However, we do not expect these funds will be sufficient to complete the clinical development of, or commercialize, any of our product candidates or programs. We have based these estimates on assumptions that may prove to be wrong, and we could use our available capital resources sooner than we expect.

We believe opportunities may exist from time to time to expand our current business through acquisitions of complementary companies, products or technologies. While we have no current agreements, commitments or understandings for any specific acquisitions at this time, we may also use a portion of the net proceeds for these purposes.

This expected use of our existing cash and net proceeds from this offering represents our intentions based upon our current plans and business conditions, which could change in the future as our plans and business conditions evolve. The amounts and timing of our actual expenditures may vary significantly depending on numerous factors, including the progress of our development, the status of and results from clinical trials and the timing and

outcome of any regulatory submissions, as well as any collaborations that we may enter into with third parties for our drug candidates, and any unforeseen cash needs. As a result, our management will have broad discretion in the application of the net proceeds from this offering, and investors will be relying on the judgment of our management regarding the application of those net proceeds. Pending our use of the net proceeds from this offering, we intend to invest the net proceeds in a variety of capital preservation investments, including short-term, investment grade, interest-bearing instruments and U.S. government securities.

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.

Capitalization

The following table sets forth our cash and our capitalization as of December 31, 2021:

- on an actual basis;
- on a pro forma basis to give effect to: (1) the conversion of all outstanding shares of our preferred stock into an aggregate of shares of our common stock; and (2) the filing and effectiveness of our amended and restated certificate of incorporation upon the closing of this offering; and
- on a pro forma as adjusted basis to give effect to: (i) the pro forma adjustments described above; and (ii) our issuance and sale of shares of common stock in this offering at an assumed initial public offering price of $ per share, which is the midpoint of the price range set forth on the cover page of this prospectus, after deducting the estimated underwriting discounts and estimated offering expenses payable by us.

This pro forma as adjusted information is illustrative only and will depend on the actual initial public offering price and other terms of this offering determined at pricing. Our capitalization following the closing of this offering will depend on the actual initial public offering price and other terms of this offering determined at pricing. You should read this information in conjunction with our financial statements and the related notes appearing at the end of this prospectus, the section of this prospectus titled “Management’s Discussion and Analysis of Financial Condition and Results of Operations” and other financial information contained in this prospectus.

	As of December 31, 2021		
	Actual	Pro Forma	Pro Forma As Adjusted
Cash	$	$	$
Convertible preferred stock, $0.0001 par value; shares authorized, issued and outstanding, actual; no shares authorized, no shares issued or outstanding, pro forma and pro forma as adjusted			
Stockholders’ (deficit) equity:			
Preferred stock, $0.0001 par value; no shares authorized, issued or outstanding, actual; shares authorized and no shares issued or outstanding, pro forma and pro forma as adjusted			
Common stock, $0.0001 par value; shares authorized, shares issued and outstanding, actual; shares authorized, shares issued and outstanding, pro forma; shares authorized, shares issued and outstanding, pro forma as adjusted			
Additional paid-in capital			
Accumulated deficit			
Total stockholders’ (deficit) equity			
Total capitalization	$	$	$

Each $1.00 increase (decrease) in the assumed initial public offering price of $ per share, which is the midpoint of the price range set forth on the cover page of this prospectus, would increase (decrease) the pro forma as adjusted amount of each of cash, additional paid in capital, total stockholders’ equity and total capitalization by approximately $ million, assuming that the number of shares offered by us, as set

forth on the cover page of this prospectus, remains the same and after deducting estimated underwriting discounts and commissions and estimated offering expenses payable by us. Similarly, each increase (decrease) of 1.0 million shares in the number of shares offered by us at the assumed initial public offering price per share of $ per share, which is the midpoint of the price range set forth on the cover page of this prospectus, would increase (decrease) the pro forma as adjusted amount of each of cash, additional paid in capital, total stockholders' equity and total capitalization by approximately $ million.

The number of shares of our common stock outstanding as of December 31, 2021 in the table above excludes:

- shares of our common stock issuable upon the exercise of options under the Existing Plan, granted subsequent to December 31, 2021, at a weighted-average exercise price of $ per share;
- shares of our common stock reserved for future issuance under the Existing Plan, which shares will cease to be available for issuance at the time the 2022 Plan becomes effective and will be added to, and become available for issuance under, the 2022 Plan;
- shares of our common stock reserved for future issuance under the 2022 Plan, which will become effective in connection with this offering, as well as any automatic increases in the number of shares of common stock reserved for future issuance under the 2022 Plan; and
- shares of our common stock reserved for future issuance under the 2022 Employee Stock Purchase Plan, or ESPP, which will become effective in connection with this offering, as well as any automatic increases in the number of shares of common stock reserved for future issuance under the ESPP.

Dilution

If you invest in our common stock in this offering, your ownership interest will be immediately diluted to the extent of the difference between the initial public offering price per share and the pro forma as adjusted net tangible book value per share of our common stock after this offering.

As of December 31, 2021, we had a historical net tangible book value (deficit) of $ million, or $ per share of common stock. Our historical net tangible book value (deficit) per share represents total tangible assets less total liabilities, divided by the number of shares of our common stock outstanding as of December 31, 2020.

Our pro forma net tangible book value (deficit) as of December 31, 2021 was $ million, or $ per share of our common stock. Pro forma net tangible book value represents the amount of our total tangible assets less our total liabilities, after giving effect to the conversion of all outstanding shares of our convertible preferred stock into an aggregate of shares of our common stock, as if such conversion had occurred on December 31, 2021. Pro forma net tangible book value per share represents pro forma net tangible book value divided by the total number of shares outstanding as of December 31, 2021, after giving effect to the pro forma adjustments described above.

After giving further effect to the sale of shares of common stock in this offering at an assumed initial public offering price of $ per share, which is the midpoint of the price range set forth on the cover page of this prospectus, and after deducting the estimated underwriting discounts and commissions and estimated offering expenses payable by us, our pro forma as adjusted net tangible book value as of December 31, 2021 would have been $ million, or $ per share. This amount represents an immediate increase in pro forma net tangible book value of $ per share to our existing stockholders and immediate dilution of $ per share to new investors in this offering. We determine dilution by subtracting the pro forma as adjusted net tangible book value per share after this offering from the amount of cash that a new investor paid for a share of common stock in this offering.

The following table illustrates this dilution:

Assumed initial public offering price per share		$
Historical net tangible book value (deficit) per share as of December 31, 2021	$	
Pro forma increase in net tangible book value (deficit) per share attributable to the pro forma transactions described above		
Pro forma net tangible book value (deficit) per share as of December 31, 2021		
Increase in pro forma as adjusted net tangible book value per share attributable to new investors participating in this offering		
Pro forma as adjusted net tangible book value per share after this offering		
Dilution per share to new investors participating in this offering		$

The dilution information discussed above is illustrative only and will change based on the actual initial public offering price and other terms of this offering determined at pricing. Each $1.00 increase (decrease) in the assumed initial public offering price of $ per share, which is the midpoint of the price range set forth on the cover page of this prospectus, would increase (decrease) the pro forma as adjusted net tangible book value per share after this offering by approximately $, and dilution in pro forma as adjusted net tangible book value per share to new investors by approximately $, assuming that the number of shares offered by us, as set forth on the cover page of this prospectus, remains the same and after deducting estimated underwriting

discounts and commissions and estimated offering expenses payable by us. An increase of 1.0 million shares in the number of shares we are offering would increase the pro forma as adjusted net tangible book value per share after this offering by $ and decrease the dilution per share to new investors participating in this offering by $, assuming no change in the assumed initial public offering price per share and after deducting the estimated underwriting discounts and commissions. A decrease of 1.0 million shares in the number of shares we are offering would decrease the pro forma as adjusted net tangible book value per share after this offering by $ and increase the dilution per share to new investors participating in this offering by $, assuming no change in the assumed initial public offering price per share and after deducting the estimated underwriting discounts and commissions.

If the underwriters exercise their option to purchase additional shares of our common stock in full, the pro forma as adjusted net tangible book value after this offering would be $ per share, the increase in pro forma as adjusted net tangible book value per share would be $ and the dilution per share to new investors would be $ per share, in each case assuming an initial public offering price of $ per share, which is the midpoint of the price range set forth on the cover page of this prospectus.

The following table summarizes, as of December 31, 2021, on the pro forma as adjusted basis described above, the differences between the number of shares purchased from us, the total consideration paid to us in cash and the average price per share that existing stockholders and new investors paid for such shares. The calculation below is based on an assumed initial public offering price of $ per share, which is the midpoint of the price range set forth on the cover page of this prospectus, before deducting the estimated underwriting discounts and commissions and estimated offering expenses payable by us.

	Total Shares		Total Consideration		Weighted-Average Price Per Share
	Number	Percent	Amount	Percent	
Existing stockholders		%	$	%	$
New investors					$
Total		100%		$ 100%	

The foregoing tables and calculations are based on the number of shares of our common stock outstanding as of December 31, 2021, after giving effect to the conversion of all outstanding shares of our convertible preferred stock into an aggregate of shares of common stock, and excludes:

- shares of our common stock issuable upon the exercise of options under the Existing Plan, granted subsequent to December 31, 2021, at a weighted-average exercise price of $ per share;
- shares of our common stock reserved for future issuance as of December 31, 2021 under the Existing Plan, which shares will cease to be available for issuance at the time the 2022 Plan becomes effective and will be added to, and become available for issuance under, the 2022 Plan;
- shares of our common stock reserved for future issuance under the 2022 Plan, which will become effective in connection with this offering, as well as any automatic increases in the number of shares of common stock reserved for future issuance under the 2022 Plan; and
- shares of our common stock reserved for future issuance under the ESPP, which will become effective in connection with this offering, as well as any automatic increases in the number of shares of common stock reserved for future issuance under the ESPP.

To the extent that stock options are exercised, new stock options are issued under our equity incentive plan or we issue additional shares of common stock in the future, there will be further dilution to investors participating in

this offering. In addition, we may choose to raise additional capital because of market conditions or strategic considerations, even if we believe that we have sufficient funds for our current or future operating plans. If we raise additional capital through the sale of equity or convertible debt securities, the issuance of these securities could result in further dilution to our stockholders.

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 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 gene therapy company focused on addressing some of the most devastating genetically defined cardiovascular and central nervous system diseases affecting both larger-rare and prevalent patient populations. We are advancing a deep and diverse pipeline of AAV-based gene therapy candidates utilizing our modular approach that integrates clinically validated technology, a disease area strategy targeting defined patient sub-populations most likely to benefit from our gene therapy candidates, and high-quality, scalable manufacturing, which is designed to overcome many of the challenges facing the field of gene therapy.

We expect to initiate a Phase 1/2 clinical trial in mid-2022 for our most advanced cardiovascular program, LX2006 for the treatment of patients with CNS FA cardiomyopathy, and we expect to report initial biomarker data by the end of 2022. Our lead product candidate, LX1001, is in an ongoing Phase 1/2 trial for the treatment of *APOE4*-associated Alzheimer's disease, and we have initially observed an increase in expression levels of the protective protein, APOE2, and a reduction in core Alzheimer's disease biomarkers in the low-dose cohort of this ongoing trial. We expect to report initial biomarker data from the mid-dose cohort by the end of 2022. Utilizing a step-wise, capital-efficient development approach, we are leveraging early proof-of-concept functional and biomarker data to build a pipeline of gene therapy programs targeting cardiovascular indications and Alzheimer's disease with genetically defined populations.

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. As of December 31, 2021, we had $ million of cash. As of December 31, 2021, we raised aggregate gross proceeds of $183.7 million from the sale of equity securities.

- On May 28, 2020 through August 11, 2020, we entered into two convertible securities purchase agreements, or the Convertible Securities Agreements, for gross proceeds of $2.6 million.
- In November 2020, we raised $30.0 million gross proceeds from the issuance of 29,999,996 shares of Series A convertible preferred stock at a purchase price of $1.00 per share. In July 2021, we raised $51.0 million gross proceeds from the additional issuance of 50,999,997 shares of Series A convertible preferred stock at a purchase price of $1.00 per share.
- In August 2021, we raised $100.1 million gross proceeds from the issuance of 58,157,823 shares of Series B convertible preferred stock at a purchase price of $1.72049 per share.

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, 2020 and 2021 were $5.2 million and $ million, respectively, and our accumulated deficit was $5.5 million at December 31, 2020. 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 will 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 research and development of LX2006, LX1001, and LX1004, and other product candidates;
- initiate preclinical studies and clinical trials for any additional product candidates, including LX2020, LX2021, LX2022, LX1020 and LX1021, that we may pursue in the future;
- 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.

We will not generate revenue from product sales unless and until we successfully complete clinical development and obtain regulatory approval for one or more of our product candidates. If we obtain regulatory approval for any of our product candidates, we expect to incur significant expenses related to product sales, marketing and distribution to the extent that such sales, marketing and distribution are not the responsibility of potential collaborators. We will require substantial additional funding to develop our product candidates and support our continuing operations. We may also require additional capital to pursue in-licenses or acquisitions of other product candidates. We may also require additional capital if we choose to pursue an in-house manufacturing strategy. Further, following the completion of this offering we expect to incur additional costs associated with operating as a public company. We also expect to increase the size of our administrative function to support the growth of our business. Our net losses may fluctuate significantly from quarter-to-quarter and year-to-year, depending on the timing of our clinical trials and our expenses related to other research and development activities.

Until such time that we can generate significant revenue from product sales or other sources, we expect to finance our operations through the sale of equity, debt financings or other capital sources, which could include proceeds from potential collaborations, strategic partnerships or marketing, distribution, licensing or other strategic arrangements with third parties, or from grants. We may be unable to raise additional funds or to enter into such agreements or arrangements on favorable terms, or at all. If we fail to raise capital or enter into such agreements 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.

Our failure to obtain sufficient funds with acceptable terms could have a material adverse effect on our business, results of operations or financial condition, including requiring us to have to delay, reduce or eliminate our product development or future commercialization efforts. Because of the numerous risks and uncertainties associated with pharmaceutical product development, we are unable to accurately predict the amount of increased expenses or timing, or if we will be able to achieve or maintain profitability. If we fail to become profitable or are unable to sustain profitability on a continuing basis, then we may be unable to continue our operations at planned levels and be forced to reduce or terminate our operations. We cannot provide assurance that we will ever be profitable or generate positive cash flow from operating activities.

Impact of COVID-19 on our operations

In March 2020, the World Health Organization declared the outbreak of COVID-19 a global pandemic. We are subject to a number of risks associated with the COVID-19 global pandemic, including potential delays associated with our ongoing preclinical studies and clinical trials. COVID-19 may have an adverse impact on our operations, supply chains and distribution systems or those of our third-party vendors and collaborators, and increase expenses, including as a result of impacts associated with preventive and precautionary measures that are being taken, such as restrictions on travel, quarantine polices and social distancing. We and our third-party vendors and collaborators may experience disruptions in supply of items that are essential for our research and development activities. In addition, the spread of COVID-19 has disrupted global healthcare and healthcare regulatory systems which could divert healthcare resources away from, or materially delay, FDA approval and approval by other health authorities worldwide with respect to our product candidates. Furthermore, our ongoing and anticipated clinical trials may be negatively affected by the COVID-19 outbreak. Site initiation, patient enrollment and patient follow-up visits may be delayed, for example, due to prioritization of hospital resources toward the COVID-19 outbreak, travel restrictions, the inability to access sites for initiation and monitoring, and difficulties recruiting or retaining patients in our planned clinical trials. We have experienced delays in patient enrollment and certain follow up visits in the Phase 1/2 clinical trial of LX1001. We cannot at this time predict the specific extent, duration, or full impact that the COVID-19 outbreak will have on our financial condition and operations.

Exclusive license agreement with Cornell University

On May 27, 2020, we entered into two exclusive license agreements with Weill Cornell Medicine. We refer to these agreements as the Cornell First License Agreement and the Cornell Second License Agreement, and collectively as the Cornell License Agreements. The Cornell First License Agreement is for the in-license of technology related to portfolios for *APOE*, Alzheimer's disease, and Anti-Tau, although our license is not restricted by such indications and it includes assignment to us of Cornell University's IND for the use of AAVrh10.hAPOE vector to treat *APOE4* homozygous patients who are at risk for or have Alzheimer's disease. The Cornell Second License Agreement is for the in-license of technology related to portfolios for CLN2 Batten disease and Friedreich's ataxia although our license is not restricted by such indications, and it includes

assignment to us of Cornell University's IND for the use of AAVrh.10CUhCLN2 to treat children with CLN2 Batten disease. Through the Cornell License Agreements, we gain access to a portfolio of inventions, patent rights, technology, and licensed methods that we plan to develop. Under the terms of the Cornell License Agreements, we assumed all worldwide development and commercialization activities with respect to the licensed technology.

As initial consideration for the Cornell License Agreements, 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, for each of the Cornell First License Agreement and Cornell Second License Agreement. In November 2020, the $1.3 million of convertible preferred securities were cancelled in exchange for 1,337,610 shares of Series A convertible preferred stock. As additional consideration, we are required to pay Cornell University 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 upon the achievement of specific clinical and regulatory milestones under the Cornell Second License Agreement. Upon submitting our IND for LX2006 to the FDA, we achieved the first clinical milestone under the Cornell Second License Agreement, and we will pay $0.1 milion to Cornell University in the first quarter of 2022 in connection with this milestone. We are also required to pay Cornell University a flat royalty in the mid-single-digits based on net sales of the products covered by the licenses, subject to certain adjustments.

See the sections entitled "Business—First License Agreement with Cornell University" and "—Second License Agreement with Cornell University" as well as Note 9 to the financial statements appearing elsewhere in this prospectus for more information on the Cornell License Agreements.

Exclusive license agreement with Adverum Biotechnologies Inc.

On January 25, 2021, we entered into an exclusive license agreement, or the Adverum Agreement, with Adverum Biotechnologies, Inc., or Adverum, to in-license materials and technology related to the treatment of FA cardiomyopathy. In connection with the Adverum Agreement, we gained access to a portfolio of inventions, patent rights, technology, and licensed methods that we will continue to develop, and we have assumed all worldwide development and commercialization activities with respect to this portfolio. Under the Adverum Agreement, we paid Adverum a $7.5 million upfront payment in 2021. We are obligated to pay Adverum up to $17.5 million upon the achievement of specified development and regulatory milestones and up to $49.0 million in commercialization and sales milestones for the products. We are obligated to pay Adverum tiered royalties ranging from a rate in the high single-digits to 10% 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. No regulatory or commercial milestones have been achieved to date under the Adverum Agreement.

See the section entitled "Business–License Agreement with Adverum" and Note 9 to the financial statements appearing elsewhere in this prospectus for more information on the Adverum Agreement.

Research collaboration agreement with Weill Cornell Medicine

On February 2, 2021, we entered into a Research Collaboration Agreement, or the Cornell Collaboration Agreement, with Weill Cornell Medicine, in conjunction with the Cornell License Agreements entered on May 27, 2020. We committed to fund scientific research at Weill Cornell Medicine related to the intellectual property licensed to us pursuant to the Cornell License Agreements.

Under the terms of the Cornell Collaboration Agreement, we will pay Weill Cornell Medicine a minimum of $1.0 million in three consecutive years, for an aggregate of at least $3.0 million. During 2021, we paid Weill

Cornell Medicine $2.3 million, and we are required to pay Weill Cornell Medicine additional consideration of $2.0 million for continuing research and development activities over the following two years. With respect to each Weill Cornell Medicine invention, joint invention, and related joint results for which an Improvement, as defined in the Cornell Collaboration Agreement, applies and for which we have made an election to amend either of the Cornell License Agreements, we have the first option to negotiate for a royalty-bearing, worldwide license to such Improvement.

See the Note 9 to the financial statements appearing elsewhere in this prospectus for more information on the Cornell Collaboration Agreement.

Stelios Therapeutics Inc. acquisition

On July 16, 2021, we purchased all of the issued and outstanding capital stock of Stelios Therapeutics, Inc., or Stelios, for initial cash consideration of $7.0 million, with payments of up to an additional $20.5 million due upon the achievement of certain milestones related to the acquired assets. As of December 31, 2021, no milestones were achieved related to the Stelios assets.

We accounted for the acquisition of Stelios as an asset acquisition pursuant to Financial Accounting Standards Board Accounting Standards Codification Section 805, *Business Combinations*. We acquired in-process research and development, or IPR&D, assets from Stelios related to ARVC and TNNI3-associated hypertrophic cardiomyopathy programs. 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.

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

On October 4, 2021, we entered into an exclusive worldwide license agreement, or the UCSD Agreement, with the Regents of UCSD to in-license materials and intellectual property related to a gene therapy for ARVC. In connection with the UCSD Agreement, we gained access to inventions, patent rights, technology, and licensed methods that we plan to develop, and we have assumed all worldwide development and commercialization activities with respect to the licensed technology. The UCSD Agreement requires us to pay a one-time up-front non-refundable cash fee of $20,000, development and commercialization milestones in aggregate up to $5.9 million, and low- to mid-single digit royalties based on aggregate net sales. During 2021, we did not make any payments pursuant to the UCSD Agreement.

See Note 9 to the financial statements appearing elsewhere in this prospectus for more information on the UCSD Agreement.

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 LX1004, 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; 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 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 advance LX2006, LX1001, LX1004, 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. Our future expenses may vary significantly each period based on factors such as:

- the timing and progress of preclinical and clinical development activities;
- the number and scope of preclinical and clinical programs we decide to pursue;
- our ability to maintain our current research and development programs and to establish new ones;
- establishing an appropriate safety profile with investigational new IND-enabling toxicology studies;
- successful patient enrollment in, and the initiation and completion of, our ongoing and planned clinical trials;
- the successful completion of clinical trials with safety, tolerability and efficacy profiles that are satisfactory to the FDA or any comparable foreign regulatory authority;
- receipt of regulatory approvals from applicable regulatory authorities;
- the timing, receipt and terms of any marketing approvals from applicable regulatory authorities;
- our ability to establish licensing or collaboration arrangements;
- the performance of our current and potential future collaborators;
- establishing commercial manufacturing capabilities or making arrangements with third-party manufacturers;
- development and timely delivery of commercial-grade therapeutics that can be used in our planned clinical trials and for commercial launch;
- commercializing our product candidates, if approved, whether alone or in collaboration with others;
- obtaining, maintaining, defending and enforcing patent claims and other intellectual property rights;
- maintaining a continued acceptable safety profiles of our therapeutics following approval;
- hiring and retaining key research and development personnel; and
- the effects of COVID-19 to our research and development employees, contractors and those who may participate in our studies.

Any changes in the outcome of any of these variables could mean a significant change in the costs and timing associated with the development of our product candidates. We may never obtain regulatory approval for any

of our product candidates, and, even if we do, drug commercialization takes several years and involves substantial cost.

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 in the foreseeable future as we increase our headcount to support the continued research and development of our programs and the growth of our business. We also anticipate incurring additional expenses associated with operating as a public company, including increased expenses related to audit, legal, regulatory, and tax-related services associated with maintaining compliance with the rules and regulations of the Securities and Exchange Commission, or SEC, and standards applicable to companies listed on a national securities exchange, additional insurance expenses, investor relations activities and other administrative and professional services.

Other income (expense)

Loss on extinguishment of notes

On May 28, 2020 and August 11, 2020, we entered into the Convertible Securities Agreements with numerous investors to issue notes with an aggregate principal of $2.6 million. We also entered into a third convertible securities purchase agreement on May 27, 2020 to retire a $1.3 million obligation with Cornell University. This third agreement with the Convertible Securities Agreements are collectively referred to as the Notes. The Notes included an annual compound interest rate of 6% and matured on the respective fifth anniversary of each Note, unless earlier converted. In consideration for the Notes, we received $2.6 million in cash and retired a $1.3 million obligation under the Cornell License Agreements. The Notes were issued on May 27, 2020 and August 11, 2020, contained a conversion option feature that met the definition of a derivative and were required to be bifurcated. The initial fair value of the conversion option feature of $0.7 million was recorded as a separate liability, and as a reduction to the carrying value of the Notes.

In November 2020, in connection with our issuance and sale of Series A convertible preferred stock, all of the outstanding principal and interest under the Notes and the convertible preferred securities issued to Cornell University were converted into shares of Series A convertible preferred stock.

Other income

In 2020, we sold shares of our Series A convertible preferred stock under a purchase agreement that included provisions that obligated investors to participate in two subsequent funding events, subject to satisfaction of certain conditions set forth therein. Each of the two funding events represented a separate tranche obligation as the conditions for each tranche of funding could be met separately and an investor could transfer part of any rights under the agreement, including the right/obligation to participate in an individual tranche. We refer to the two funding obligations collectively as the Preferred Stock Tranche Obligation.

Other income includes the change in the fair value of the Preferred Stock Tranche Obligation, which was settled by us in July 2021.

Interest expense

Interest expense primarily consists of the interest on our Notes.

Income taxes

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 net operating loss, or NOL, carryforwards and tax credits will not be realized. As of December 31, 2021, we had federal NOL carryforwards of approximately $ million and state NOL carryforwards of approximately $ million which may be available to offset future taxable income and begin to expire in . The total federal NOL of $ million are not subject to expiration. As of December 31, 2021, we also had state tax research and development credit carryforwards of approximately $ to offset future tax liabilities, which begin to expire in . As of December 31, 2021, we had no federal tax research and development credit carryforwards. We have recorded a full valuation allowance against our net deferred tax assets at December 31, 2021. As of December 31, 2021, we had no unrecognized tax benefits.

Results of operations

Comparison of the years ended December 31, 2020 and 2021

The following table summarizes our results of operations for the years ended December 31, 2020 and 2021:

	Years ended December 31,		
	2020	2021	Change
Grant revenue	$ 518,476	$	$
Operating expenses:			
Research and development	4,318,765		
General and administrative	787,499		
Total operating expenses	5,106,264		
Operating loss	(4,587,788)		
Other income (expense), net			
Loss on extinguishment of Notes	(422,091)		
Other income	4,241		
Interest expense	(147,150)		
Total other income (expense), net	(565,000)		
Loss from operations before income taxes	(5,152,788)		
Income taxes	—		
Net loss and comprehensive loss	$ (5,152,788)	$	$

Research and development expenses

The following table summarizes our research and development expenses incurred by program for the years ended December 31, 2020 and 2021:

	Years ended December 31,		
	2020	2021	Change
Direct external research and development expenses by program:			
LX2006	$ —	$	$
LX1001	919,129		
LX1004	—		
Other programs	—		
Unallocated research and development expenses:			
Professional fees and other	1,603,047		
Licenses fees and other	1,796,589		
Total research and development expenses	$ 4,318,765	$	$

Research and development expenses were $4.3 million for the year ended December 31, 2020 compared to $ million for the year ended December 31, 2021. The increase of $ million was primarily due to .

Direct external research and development expenses for LX2006 was $ million for the year ended December 31, 2021, which was primarily attributable to . We did not incur significant direct, external research and development expense for LX2006 for the year ended December 31, 2020.

Direct external research and development expense for LX1001 was $0.9 million for the year ended December 31, 2020, compared to $ million for the year ended December 31, 2021. The increase of $ million was primarily due to .

Direct external research and development expenses for LX1004 was $ million for the year ended December 31, 2021, which was primarily attributable to . We did not incur significant direct, external research and development expense for LX1004 for the year ended December 31, 2020.

Direct external research and development expenses for our other programs was $ million for the year ended December 31, 2021, which was primarily attributable to . We did not incur significant direct, external research and development expense for other programs for the year ended December 31, 2020.

Unallocated research and development expenses were $3.4 million for the year ended December 31, 2020, of which $1.6 million was attributable to professional fees and similar expenses and $1.8 million was related to license fees and similar expenses. Unallocated research and development expenses were $ million for the year ended December 31, 2021, of which $ million was attributable to professional fees and other and $ million was related to license fees and other. The increase of $ million in unallocated research and development expenses was primarily due to .

General and administrative expenses

General and administrative expenses were $0.8 million for the year ended December 31, 2020 compared to $ million for the year ended December 31, 2021. The increase of $ million was primarily due to .

Loss on extinguishment of notes

In November 2020, in connection with our issuance and sale of Series A convertible preferred stock, all $3.9 million of outstanding principal and $0.1 million of accrued interest was converted into 4,495,719 shares of our Series A convertible preferred stock at a price ranging between 85-100% of $1.00 per share depending on the investor. See Note 5 to our financial statements appearing elsewhere in this prospectus for additional information.

We accounted for the conversion of the Notes as a debt extinguishment and recognized a loss on extinguishment of notes of $1.2 million within other income (expense), net in the statement of operations. The loss on extinguishment was calculated as the difference between the carrying value of the Notes, net of the unamortized debt discount of $3.3 million, and the $4.5 million fair value of the 4,495,719 shares of Series A convertible preferred stock issued to settle the Notes. Upon conversion, the loss on extinguishment of notes was offset by the extinguishment of the conversion option feature of $0.8 million. See Note 3 and Note 4 to our financial statements appearing elsewhere in this prospectus for additional information.

Interest expense

We recognized interest expense of $0.1 million for the year ended December 31, 2020 related to interest accrued on the Notes prior to their extinguishment in November 2020. We did not recognize interest expense during the year ended December 31, 2021.

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, 2021, we have funded our operations with net proceeds from sales of our convertible preferred stock and Convertible Securities Agreements of $183.7 million. As of December 31, 2021, we had cash of $ million.

Based on our current operating plan, we expect the net proceeds from this offering, together with our existing cash, will be sufficient to fund our planned operating expenses and capital expenditure requirements through . 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.” Our development plans may be revised, and our estimated use of proceeds may be impacted.

Cash flows

The following table summarizes our sources and uses of cash for each of the periods presented:

	Years ended December 31,	
	2020	**2021**
Net cash used in operating activities	$ (3,133,396)	$
Net cash used in investing activities	—	
Net cash provided by financing activities	32,378,745	
Net increase in cash	$ 29,245,349	$

Operating activities

During the year ended December 31, 2020, net cash used in operating activities consisted primarily of our net loss of $5.2 million, partially offset by (i) $1.3 million retirement of the exclusive license agreement with Cornell University, (ii) $0.4 million loss on extinguishment of the Notes, (iii) $0.1 million accrued interest expense and (iv) $0.1 million change on operating assets and liabilities.

Investing activities

During the year ended December 31, 2020, there were no cash flows from investing activities.

Financing activities

During the year ended December 31, 2020, net cash provided by financing activities consisted primarily of $30.0 million gross proceeds from the issuance of 29,999,996 shares of Series A convertible preferred stock at a purchase price of $1.00 per share, offset by $0.2 million of issuance costs, and $2.6 million gross proceeds from the issuance of Convertible Securities Agreements.

Our primary uses of cash are to fund our 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, following the completion of this offering, we expect to incur additional costs associated with operating as a public company. Accordingly, 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;

- 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, including financial and reporting systems.

Identifying potential product candidates and conducting preclinical studies and clinical trials is a time-consuming, expensive and uncertain process that takes years to complete, and we may never generate the necessary data or results required to obtain regulatory approval and commercialize our product candidates. In addition, our product candidates, if approved, may not achieve commercial success. Our commercial revenues, if any, will be derived from sales of products that we do not expect to be commercially available for many years, if at all. Accordingly, we will need to continue to rely on additional financing to achieve our business objectives.

Until such time, if ever, as we can generate significant revenue from product sales or other sources, we expect to finance our operations through the sale of equity, debt financings, or other capital sources, which could include proceeds from potential collaborations, strategic partnerships or marketing, distribution, licensing or other similar arrangements with third parties. However, we may be unable to raise additional funds or enter into such agreements or arrangements on favorable terms, or at all. Market volatility resulting from the COVID-19 pandemic or other factors could also adversely impact our ability to access capital as and when needed. To the extent that we raise additional capital through the sale of equity or convertible debt securities, your ownership interest will be diluted, and the terms of these securities may include liquidation or other preferences that adversely affect your rights as a common stockholder. Any future 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, selling or licensing our assets, making capital expenditures, declaring dividends or encumbering our assets to secure future indebtedness. Such restrictions could adversely impact our ability to conduct our operations and execute our business plan.

If we raise funds through collaborations, strategic alliances or licensing arrangements with third parties, we may have to relinquish valuable rights to our technologies, future revenue streams, research programs or product candidates or to grant licenses on terms that may not be favorable to us. If we fail to raise capital or enter into such agreements 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.

Contractual obligations and commitments

The following table summarizes our contractual obligations and commitments as of December 31, 2021:

($ in thousands)		**Payments Due by Period**			
Description	**Total**	**Less than 1 year**	**1 – 3 years**	**3—5 years**	**More than 5 years**
Operating leases	$	$	$	$	$
Total	$	$	$	$	$

Apart from the contracts with payment commitments that we have reflected in the table above, we have entered into contracts in the normal course of business with CROs, CMOs and other third parties for preclinical research studies and testing, clinical trials and manufacturing services. These contracts do not contain any minimum purchase commitments and are cancelable by us upon prior notice and, as a result, are not included in the table of contractual obligations and commitments above. Payments due upon cancellation consist only of payments for services provided and expenses incurred, including non-cancelable obligations of our service providers, up to the date of cancellation.

We may in the future incur potential royalty payments under license and collaboration agreements we have entered into with various entities, pursuant to which we have in-licensed certain intellectual property. Due to the uncertainty of the achievement and timing of the events requiring payment under these agreements, the amounts to be paid by us are not fixed or determinable at this time and are excluded from the table above.

Off-balance sheet arrangements

We did not have during the periods presented, and we do not currently have, any off-balance sheet arrangements, as defined in the rules and regulations of the SEC.

Critical accounting policies and significant judgements and estimates

Our management's discussion and analysis of our financial condition and results of operations are based on our financial statements, which have been prepared in accordance with U.S. generally accepted accounting principles, or U.S. GAAP. The preparation of our 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 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 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 financial statements.

Research and development

As part of the process of preparing our 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 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
- 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

As there has been no public market for our common stock to date, the estimated fair value of our common stock has been 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 $0.22 as of November 20, 2020, $0.46 as of August 11, 2021 and $1.38 as of December 17, 2021. 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;

- 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 initial public offering, or 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.

The assumptions underlying these valuations represented management's best estimate, which involved inherent uncertainties and the application of management's judgment. As a result, if we had used significantly different assumptions or estimates, the fair value of our common stock and our stock-based compensation expense could have been materially different.

Once a public trading market for our common stock has been established in connection with the completion of this offering, it will no longer be necessary for our board of directors to estimate the fair value of our common stock in connection with our accounting for granted stock options and other such awards we may grant, as the fair value of our common stock will be determined based on the quoted market price of our common stock.

Option grants

The following table summarizes by grant date the number of shares subject to options granted since February 12, 2021, the date we adopted the 2021 Equity Incentive Plan, or the Existing Plan, through December 31, 2021, the per share exercise price of the options, the per share fair value of our common stock on each grant date and the per share estimated fair value of the options:

Grant Date	Number of Shares Subject to Options Granted	Per Share Exercise Price of Options	Per Share Fair Value of Common Stock on Grant Date	Per Share Estimated Fair Value of Options
February 16, 2021	5,304,916	$ 0.22	$ 0.22	$ 0.13
March 9, 2021	1,791,822	$ 0.22	$ 0.22	$ 0.13
March 19, 2021	320,461	$ 0.22	$ 0.22	$ 0.13
March 22, 2021	487,350	$ 0.22	$ 0.22	$ 0.13
April 5, 2021	225,928	$ 0.22	$ 0.22	$ 0.13
May 12, 2021	113,128	$ 0.22	$ 0.22	$ 0.13
May 14, 2021	18,000	$ 0.22	$ 0.22	$ 0.13
May 17, 2021	160,528	$ 0.22	$ 0.22	$ 0.13
November 4, 2021	416,184	$ 0.46	$ 1.38(1)	$ 0.20
November 9, 2021	461,314	$ 0.46	$ 1.38(1)	$ 0.20
November 15, 2021	11,679,474	$ 0.46	$ 1.38(1)	$ 0.20

(1) At the time of the option grants approved on November 4, November 9 and November 15, 2021, our board of directors had determined that the fair value of our common stock was $0.46 per share based in part on a third-party valuation of our common stock as of August 2021. However, in December 2021, we performed a retrospective fair value assessment and concluded that the fair value of our common stock underlying the stock options that were granted on November 4, November 9 and November 15, 2021 was $1.38 per share for accounting purposes. The reassessed value was based, in part, upon a third-party valuation of our common stock prepared on a retrospective basis as of December 17, 2021.

Valuation of the preferred stock tranche obligation

The Preferred Stock Tranche Obligation was recorded at fair value at the issuance date and a portion of the proceeds from the first closing equal to that amount was allocated to it. At November 20, 2020, the fair value of

the Preferred Stock Tranche Obligation was valued at $3.0 million at a value per share equivalent to $0.05 and $0.07 for the second and third closings, respectively. The Preferred Stock Tranche Obligation was valued using a probability-weighted present value model. The valuation model considered the probability of closing each tranche, the estimated future value of the Series A convertible preferred stock to be issued at each closing and the investment required at each closing. Future values were converted to present value using a discount rate appropriate for probability-adjusted cash flows.

In July 2021, our board of directors and the holders of a majority of our then-outstanding Series A convertible preferred stock voted to consummate the two subsequent funding events, resulting in the issuance of 50,999,997 shares of Series A convertible preferred stock for aggregate proceeds to us of $51.0 million and settlement by us of the Preferred Stock Tranche Obligation. See Note 3 to our financial statements appearing elsewhere in this prospectus for additional information.

Valuation of the conversion option liabilities

As discussed above, we issued Notes on May 27, 2020, and August 11, 2020. The Notes contained certain features that met the definition of a derivative and were required to be bifurcated. We accounted for these as a single derivative comprising all the features requiring bifurcation, which we collectively refer to as the Conversion Option Liabilities. The fair value of the derivative liability was estimated using a scenario-based analysis comparing the probability-weighted present value of the Note payoff at maturity with and without the bifurcated features. We considered possible outcomes available to the investors, including a conversion into shares of convertible preferred stock that would occur in connection with a qualified financing transaction, and a mandatory conversion into shares of common stock upon a change of control. In addition to the probabilities applied to various scenarios, the key unobservable inputs were the time to liquidity for each scenario and the discount rate. The Conversion Option Liabilities were settled during the year ended December 31, 2020. See Note 3 to our financial statements appearing elsewhere in this prospectus for additional information.

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 Securities Exchange Act of 1934, as amended, 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 intend to 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 this offering; (2) the last day of the fiscal year in which our total annual gross revenue is equal to or more than $1.07 billion; (3) the date on which we have issued more than $1 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 financial statements included elsewhere in this prospectus.

Qualitative and quantitative disclosures about market risk

Interest rate risk

As of December 31, 2020, we had cash of $29.4 million. As of December 31, 2021, we had cash of $ million. Interest income is sensitive to changes in the general level of interest rates. Our surplus cash has been invested in money market fund accounts and interest-bearing savings accounts. We have not entered into investments for trading or speculative purposes. Due to the conservative nature of our investment portfolio, which is predicated on capital preservation of investments with short-term maturities, we do not believe an immediate one percentage point change in interest rates would have a material effect on the fair market value of our portfolio, and therefore we do not expect our operating results or cash flows to be significantly affected by changes in market interest rates. As of December 31, 2020 and 2021, we had no debt outstanding. Therefore, we are not exposed to interest rate risk with respect to debt.

All of our employees and our operations are currently located in the United States. We have, from time to time, engaged with contractors or other vendors in a currency other than the U.S. dollar. To date, we have not been exposed to significant market risk related to changes in foreign currency exchange rates. Our operations may be subject to fluctuations in foreign currency exchange rates in the future.

We do not believe that inflation has had a material effect on our business, financial condition or results of operations. Our operations may be subject to inflation in the future.

Business

Overview

We are a clinical-stage gene therapy company focused on addressing some of the most devastating genetically defined cardiovascular and central nervous system diseases affecting both larger-rare and prevalent patient populations. We are advancing a deep and diverse pipeline of AAV-based gene therapy candidates utilizing our modular approach that integrates clinically validated technology, a disease area strategy targeting defined patient sub-populations most likely to benefit from our gene therapy candidates, and high-quality, scalable manufacturing, which is designed to overcome many of the challenges facing the field of gene therapy.

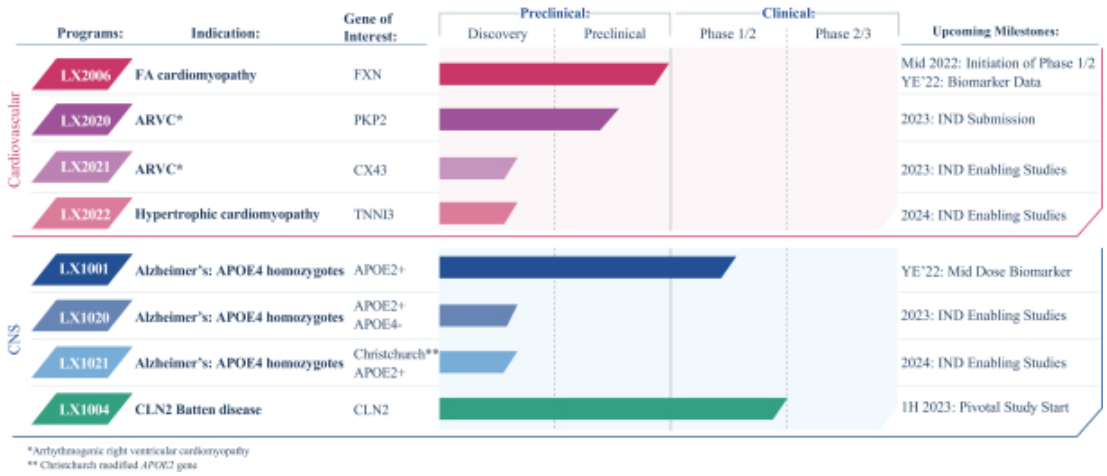
We expect to initiate a Phase 1/2 clinical trial in mid-2022 for our most advanced cardiovascular program, LX2006 for the treatment of patients with FA cardiomyopathy, and we expect to report initial biomarker data by the end of 2022. Our lead CNS product candidate, LX1001, is in an ongoing Phase 1/2 trial for the treatment of *APOE4*-associated Alzheimer's disease, and we have initially observed an increase in expression levels of the protective protein, APOE2, and a reduction in core Alzheimer's disease biomarkers in the low-dose cohort of this ongoing trial. We expect to report initial biomarker data from the mid-dose cohort by the end of 2022. Utilizing a step-wise, capital-efficient development approach, we are leveraging early proof-of-concept functional and biomarker data to build a pipeline of gene therapy programs targeting cardiovascular indications and Alzheimer's disease with genetically defined populations.

Our integrated, modular approach enables us to optimize our strategy to pursue larger-rare and prevalent genetically defined indications in specific sub-populations 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 and safety profile for our product candidate for each disease. Our most advanced cardiovascular and CNS programs use the AAVrh10 vector due to its high transduction efficiency in both myocardial cells and neurons, potentially lower toxicity given its ability to utilize lower doses compared to other well-established AAV serotypes, and lower 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-populations of patients that may be most amenable to gene therapy. These targeted indications offer the potential to show therapeutic impact through functional endpoints or biomarkers, have high unmet need and large market opportunities, have demonstrated promising preclinical data, and have organized patient advocacy groups and identifiable patient populations. We believe targeting cardiovascular and CNS diseases can be enhanced by our current approach utilizing AAVrh10 as well as ongoing discovery efforts to identify a next-generation vector technology with the best potential therapeutic profile. Finally, we continuously seek to bolster our pipeline through relationships with academic institutions, which provide us access to cutting edge gene therapy research that we will utilize in the discovery and development of next generation gene therapy candidates.

Our pipeline

We are advancing a deep and diverse pipeline of cardiovascular and CNS therapeutic programs for larger-rare and prevalent diseases. We retain exclusive worldwide development and commercialization rights to all of our product candidates and programs.



Lead cardiovascular programs

We are seeking to develop a number of disease-modifying gene therapy candidates to treat larger-rare cardiovascular diseases that have significant unmet need and no approved treatments which 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 Friedreich's ataxia and affects approximately 5,600 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 as well as restoration of cardiac function and reversal of the disease abnormalities of FA cardiomyopathy in a partial *FXN* knockout mouse model. Our IND for LX2006 was cleared by the FDA in , 2022. We expect to initiate an open-label, dose-escalation Phase 1/2 clinical trial in patients with FA cardiomyopathy in mid-2022, and we expect to report interim biomarker data by the end of 2022. The FDA has granted Rare Pediatric Disease designation and Orphan Drug designation to LX2006 for the treatment of Friedreich's ataxia.
- **LX2020** is an AAVrh10-based gene therapy candidate designed to intravenously deliver a fully functional *PKP2* gene to cardiac muscle for the treatment of ARVC caused by mutations in the *PKP2* gene. *PKP2* mutations are associated with approximately 75% of all genetic cases of ARVC, and we estimate they affect more than 70,000 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, LX2020 resulted in fewer arrhythmias and increased survival. We intend to submit an IND for LX2020 in 2023.

Lead CNS programs

We are developing a pipeline of CNS focused gene therapies that includes a portfolio of approaches to treat the genetics underlying Alzheimer's disease as well as a program designed to treat CLN2 Batten disease.

- *LX1001* is an AAVrh10-based gene therapy candidate designed to deliver via one-time administration into the 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 CNS. *APOE4* homozygous individuals 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. Currently, there are no treatments that are approved as disease-modifying for Alzheimer's disease. 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 in the United States. LX1001 is being evaluated in an ongoing open-label, dose-escalation Phase 1/2 clinical trial and we have observed a decline in Alzheimer's disease CSF biomarkers, such as total tau and phosphorylated tau, in the first two patients with 12-month data in the low-dose cohort in the trial. We have also reported data demonstrating expression of the protective APOE2 protein in all four patients in the low-dose cohort with follow-up data. We expect to report initial biomarker data from the mid-dose cohort by the end of 2022. LX1001 has been granted fast track designation by the FDA.
- *LX1004* is an AAVrh10-based gene therapy candidate designed to deliver via intracisternal injection a fully functional *CLN2* gene to the CNS in order to restore the enzyme tripeptidyl peptidase 1, or TPP1, the secreted protein that is deficient in CLN2 Batten disease patients. This disease is an autosomal recessive lysosomal storage disorder causing loss of cognitive and motor function, blindness, seizures, and ultimately death in childhood, with approximately 900 cases estimated in the United States and European Union. In a completed Phase 1/2 clinical trial, LX1004 was administered via intraparenchymal injection and demonstrated an increase in TPP1 levels leading to a slower decline of motor and language function at 18 months post treatment in treated patients compared to natural history controls. We anticipate receiving feedback from the FDA on the design of our potentially pivotal Phase 2/3 clinical trial in the second half of 2022, and we intend to initiate the clinical trial in the first half of 2023. The FDA has granted Rare Pediatric Disease designation and Orphan Drug designation to LX1004 for the treatment of CLN2 Batten disease.

Additional programs

Within our cardiovascular pipeline, we are concurrently advancing several additional AAV-based gene therapy programs to treat other targets in myocardial cells that are dysregulated in cardiomyopathy, including LX2021 designed to restore Connexin 43, or Cx43, in patients with ARVC, and LX2022 designed to treat HCM due to mutations in the *TNNI3* gene.

We are also building a portfolio of approaches to treat the genetics underlying Alzheimer's disease. Our Alzheimer's disease portfolio includes LX1020, which is designed to deliver both the protective *APOE2* gene and miRNA to suppress the expression of the deleterious *APOE4* gene, and LX1021, which is designed to deliver a Christchurch-modified *APOE2* gene. The Christchurch mutation has been shown in individuals to be effective at protecting patients against Alzheimer's disease even in the presence of significant amyloid pathology.

Our company and team

We were founded through a collaboration between the world-class gene therapy research at Weill Cornell Medicine and a team of pioneering scientists, clinicians, and business leaders with deep expertise in gene

therapy. We continue to leverage our long-standing relationship with Weill Cornell Medicine through sponsored research and development efforts to progress our cardiovascular platform technology as well as our CNS product candidates. Through our acquisition of Stelios Therapeutics, Inc., we have an ongoing collaboration with 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 CNS disorders 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.

We are led by pioneers and experts with decades of collective experience in genetic medicines, rare disease drug development, manufacturing and commercialization. Our scientific founder and Chief Scientific Advisor, Ronald G. Crystal, M.D., is a world leader in gene therapy research and development and currently serves as Professor and Chairman of Weill Cornell Medicine's Genetic Medicine Department and Director of the Belfer Gene Therapy Core Facility. Dr. Crystal has sponsored 14 cleared gene therapy IND applications and has published more than 300 papers on gene therapy. He has more than thirty years of experience with AAVs and vector design and expertise in CNS, cardiac, pulmonary and liver-mediated diseases. Our Chief Executive Officer, R. Nolan Townsend, has spent more than a decade working in global biopharmaceutical commercial organizations, including leading Pfizer's $1.6 billion international rare disease commercial business unit. Mr. Townsend also led Pfizer's U.S. rare disease commercial business unit, where he oversaw the successful U.S. launch of blockbuster rare cardiovascular product (tafamidis) Vyndaqel/Vyndamax, now a $2 billion product by revenue. He has overseen other successful rare disease product launches and been involved in business and commercial development efforts for several gene therapy programs. Dr. Jay A. Barth, our Executive Vice President and Chief Medical Officer, has more than 20 years of experience in drug development in the biopharmaceutical industry. He was formerly the Chief Medical Officer at Amicus Therapeutics, Inc. and Senior Vice President, Clinical Development, at PTC Therapeutics, Inc., where he had clinical oversight of the initial approvals of Galafold, for the treatment of Fabry disease, at Amicus Therapeutics, Inc., as well as Translarna, the first approved treatment for Duchenne muscular dystrophy, at PTC Therapeutics, Inc. Our Chief Technical Officer, Paul McCormac, Ph.D., has more than 20 years of experience and a proven track record in the field of gene therapy and biologics chemistry, manufacturing, and controls, or CMC. He previously served as Medicinal Sciences Category Lead for Pfizer Rare Disease, where he led gene therapy CMC and vector supply strategy for Pfizer's rare disease business and research units. Our Chairman, Dr. Steven Altschuler, is currently Managing Director at Ziff Capital Investments and was formerly Chairman of the gene therapy pioneer Spark Therapeutics, Inc., which was responsible for the first FDA-approved gene therapy, voretigene neparvovec-rzyl (Luxturna), and was acquired by Roche Holding AG in 2019 for $4.3 billion. Our senior leadership is supported by the rest of our management team, which has decades of experience across research, development, manufacturing and commercialization in gene therapy and rare disease both from prominent biotechnology companies and global biopharmaceutical companies that are active in the space.

Since our inception, we have raised $183.7 million in capital from premier institutional investors, including funds affiliated with D1 Capital Partners, Eventide Healthcare & Life Sciences Fund, Janus Henderson Investors, Longitude Capital, Lundbeckfonden Ventures, Omega Funds and PBM Capital.

Our strategy

We are building a fully integrated company with the potential to significantly advance gene therapy's impact on many of society's most challenging genetically defined cardiovascular and CNS diseases. Our vision is a world where gene therapies resolve the burden of disease and transform the lives of patients. The key elements of our strategy to achieve this vision are to:

- *Focus on genetically-defined cardiovascular and CNS indications of high unmet need and substantial commercial potential*. We are focused on genetically defined cardiovascular and CNS conditions where there is no currently approved treatment or where we believe our therapies will provide a meaningful improvement relative to existing standards of care. Furthermore, we seek 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 pool of approximately 5,600 FA cardiomyopathy patients in the United States, and LX2020 has the potential to address approximately 70,000 patients with ARVC caused by *PKP2* mutations. Within our CNS programs, LX1001 has the potential to address up to approximately 900,000 patients in the United States with homozygous *APOE4*-associated Alzheimer's disease.

- *Target development efforts at disease pathologies and defined sub-populations of patients 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-populations 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. In LX2020, we are targeting the *PKP2* sub-population that represents approximately 75% of the genetic ARVC cases and presents a clinical phenotype amenable to treatment. In LX1001, we are targeting homozygous *APOE4*-associated Alzheimer's disease patients, the highest risk population to develop Alzheimer's disease and the sub-population most likely to demonstrate a treatment effect from the expression of the protective APOE2 protein.

- *Pursue a step-wise and capital-efficient development approach for advancing our programs through the clinic and regulatory approval*. We are advancing our pipeline of AAV-based gene therapies for cardiovascular and CNS indications. We expect to report biomarker data from the low-dose cohort in our Phase 1/2 clinical trial of LX2006 for the treatment of FA cardiomyopathy by the end of 2022. We expect to report initial biomarker data from the mid-dose cohort in our Phase 1/2 clinical trial of LX1001 for the treatment of *APOE4*-associated Alzheimer's disease by the end of 2022. We plan to leverage key learnings from our lead programs in cardiovascular and CNS diseases to guide the development of our next-generation candidates and follow-on indications. Given that there are several targets in myocardial cells that are dysregulated in cardiomyopathy, we believe there will be readthrough to similar indications following proof of concept from our initial FA cardiomyopathy and ARVC 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-population 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 action and support further development efforts. We believe our strategy could facilitate shorter clinical trial timelines, which coupled with expedited global regulatory approval pathways, could potentially accelerate clinical development, and reduce overall development costs.

- *Expand our cardiovascular pipeline capabilities through the discovery and development of next-generation gene therapy technologies*. We have combined our robust expertise in cardiac-targeted vector design, regulatory and clinical strategy, and disease area knowledge with our deep therapeutics pipeline to establish a leading 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

cardiovascular 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 cardiovascular programs through our own efforts and in collaboration with others.

- *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.
- *Utilize a scalable, unified manufacturing platform across all current and future programs*. 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, high-potency vectors that can accommodate demand for both rare and prevalent patient populations. We are currently working with third parties for our manufacturing capabilities; however, we will evaluate building out our own cGMP-compliant manufacturing facility as, and if, we achieve further clinical success or any of our product candidates ultimately receive marketing authorization.
- *Build a fully integrated gene therapy 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.

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 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 only two AAV-based gene therapy products approved by the FDA: voretigene neparvovec-rzyl (Luxturna), for the treatment of a rare ocular condition; and onasemnogene abeparvovec-xioi (Zolgensma), for the treatment of a rare neuromuscular condition. Less than three years after its 2019 FDA approval, Zolgensma generated $1.4 billion of net product sales in 2021, highlighting the commercial viability of novel gene therapies.

Challenges associated with gene therapies

Although substantial advances have been made in the gene therapy field, numerous challenges remain that are associated with safety, efficacy and manufacturing, including:

- *High doses resulting in toxicity in systemic gene therapy*. Like many other drug modalities, higher doses of gene therapies have been associated with increased toxicity. Thus far, systemic gene therapies that are intended to treat larger tissues or organ systems (such as in neuromuscular diseases) have required higher doses to show efficacy. Adverse effects such as liver toxicity and microvascular thrombosis may arise from higher doses of gene therapy, leading to direct injury to hepatocytes and activation of aversive immune responses.
- *Delivery and biodistribution challenges in CNS gene therapy.* Gene therapies for CNS indications may cause dorsal root ganglia pathology due to a preferential expression profile in dorsal root ganglia if administered to the CNS. In addition, gene therapies administered to the CNS must overcome the issue of insufficient biodistribution to neurons and other cells of the CNS in the case of therapies that target intracellular proteins, diminishing their potential efficacy.
- *Lack of scalable, high-quality manufacturing.* Early-stage manufacture of AAV gene therapy has traditionally relied on the use of HEK adherent and suspension cell culture using plasmid transfection and subsequent ultracentrifugation-based purification. This approach involves the use of expensive plasmids and results in a long production cycle. Additional issues include low yield and quality of the material produced, including incorporation of non-transgene DNA impurities, ratio of empty-to-full capsid and efficient removal of process residuals. It has been reported that empty capsids and DNA impurities can cause innate and adaptive immune responses to AAV and even inhibit transduction by filled capsids. Furthermore, the HEK adherent and suspension cell culture approach has proven difficult to scale up.

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-populations 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-populations within selected cardiovascular and CNS indications 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, high-potency vectors that can accommodate demand for both rare and prevalent patient populations. Finally, we continuously seek to bolster our development pipeline with relationships with academic institutions, which provide us access to cutting edge gene therapy research that we will utilize in the discovery and development of next generation gene therapy candidates.

Our technology approach

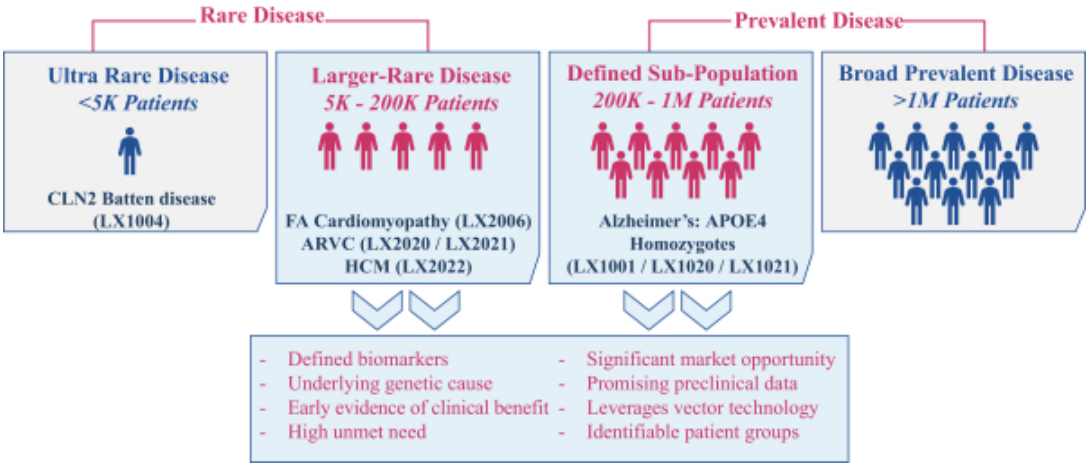
Our most advanced cardiac and CNS programs use the AAVrh10 vector due to its high transduction efficiency in both myocardial cells and neurons, higher ratio of cardiac to liver biodistribution, potentially lower toxicity given the ability to utilize lower doses, compared to other well-established AAV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

- *High Transduction Efficiency and Biodistribution.* The AAVrh10 vector has demonstrated successful transduction of myocardial cells and neurons, which we believe makes it optimal for delivery and expression of transgenes for the treatment of the cardiovascular and CNS diseases we are currently targeting. We have observed organ-specific biodistribution advantages for our AAVrh10 vector. For example, we have reported vector distribution of AAVrh10 in cardiac tissue of nonhuman primates, or NHPs, that is 1.5 to 2 times greater than for AAV9. Preclinical data from a murine model has also demonstrated that more AAVrh10 particles transduced a single brain cell than AAV9. In addition, preclinical studies 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.
- *Reduced Toxicity*. The cardiac tropism allows AAVrh10 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 that may be administered at comparatively lower doses in order to potentially reduce toxicities that have been reported in clinical trials of higher-dose gene therapies. For example, we intend to use doses of low to mid $x10^{11}$ genome copies per kilogram in our Phase 1/2 clinical trial of LX2006 for the treatment of FA cardiomyopathy, compared to doses used in other systemic gene therapy programs which can exceed 1 $x10^{14}$ genome copies per kilogram. Similarly, early data suggest a lower dorsal root ganglia toxicity profile than other commonly used vectors, making it amenable for treating CNS diseases 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 therapeutic efficacy of our product candidates.

Our disease area strategy

We employ a strategy to select targets in indications that have relatively large patient populations, high unmet need and technically feasible treatment profiles.

Our disease area strategy



We target diseases with the following characteristics:

- *Defined sub-populations in indications that may be effectively treated by gene therapy.* Our disease targets are focused on defined sub-populations of patients 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, increasing the homogeneity of our studied patient populations and increasing the potential for observing a homogeneous effect.
- *Indications with potential to demonstrate early evidence of meaningful clinical benefit.* We pursue clearly defined biomarkers, as well as functional endpoints, that can potentially provide early proof-of-mechanism and inform clinical development decisions, including the potential to pursue 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 date of treatment. In the case of LX2006 targeting FA cardiomyopathy, 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 should cure the disease.
- *Present opportunity to address high unmet medical need.* We are focused on genetically defined cardiovascular and CNS conditions where there is no currently approved treatment or where we believe our therapeutic candidates will have a meaningful improvement relative to existing standards of care.
- *Significant market opportunity*. We seek indications with significant commercial opportunity beyond those typically associated with gene therapy companies targeting rare monogenic diseases. Our cardiovascular gene therapy pipeline and our Alzheimer's disease gene therapy portfolio target either larger-rare disease populations, ranging from approximately 5,600 U.S. patients, in the case of FA cardiomyopathy, to approximately 70,000 U.S patients in the case of ARVC due to *PKP2* mutations, 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 demonstrated promising preclinical data*. We have leveraged our relationships with academic institutions including Weill Cornell Medicine and UCSD to in-license or acquire product candidates with established proof-of-concept in relevant animal models across a wide range of indications. We will continue to seek additional opportunities where promising preclinical data is available.
- *Targeted disease areas best treated by optimal vector technologies*. Our initial gene therapy candidates utilize the AAVrh10 vector due to its biodistribution profile in myocardial cells and neurons, which allow us to pursue both cardiovascular and CNS indications. For future indications, we will pursue the optimal vector technology to address the diseases of interest while ensuring sufficient preclinical or clinical validation for any novel approaches.
- *Readily identifiable patient groups*. 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 are also leveraging existing natural history studies which can help us to better define the target patient phenotypes associated with the disease.

Our manufacturing approach

We are developing gene therapy candidates for larger-rare and prevalent disease patient populations, which 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 yield, fewer empty AAV capsids, reduced incorporation of non-transgene DNA impurities and lower levels of process residuals than traditionally used plasmid HEK adherent cell culture approaches.

Our baculovirus/Sf9 expression system is designed to provide several benefits, including:

- *Ease of scalability:* potential to accommodate preclinical to large commercial indications;
- *Lower cost of goods*: utilizes a process that does not require expensive plasmids;
- *Robust yields*: potential for yields that can accommodate commercial demand for both larger-rare and prevalent patient populations;
- *Improved ratio of full capsids*: delivers a higher percentage of full capsids as compared to HEK systems;
- *Improved safety profile:*
    - reduced plasmid DNA impurities, from 15% observed in some HEK systems to 0.2% in our process;
    - cellular components are non-replicating in mammalian cells;
    - eliminates potentially immunogenic or toxic animal-derived proteins;
    - cells are grown under serum-free conditions, leading to reduced risk of contamination from animal-derived products;
- *Equivalent potency*: shows levels of potency that are similar to mammalian cell lines;

- *Aligned with industry standards*: uses traditional manufacturing infrastructure and equipment widely available in the industry; and
- *Simpler process*: it does not require an adeno-virus helper.

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 development activities or have the ability to license it and will maintain the option to transfer the process to other contract development and manufacturing companies, or 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 currently focus 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 with our pipeline to establish a leading 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 ARVC caused by mutations in the *PKP2* gene, LX2021 to treat ARVC 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 apply them to our earlier-stage cardiac programs.

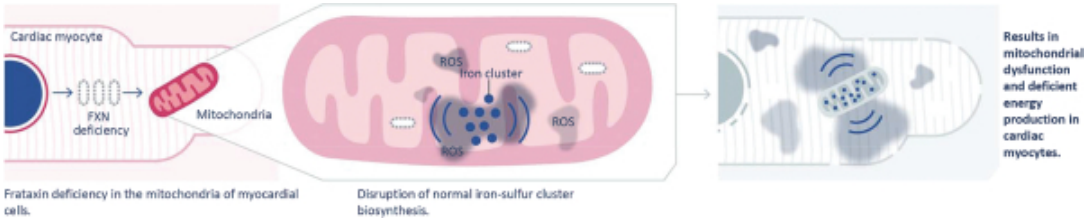
LX2006 for the treatment of FA cardiomyopathy

We are developing LX2006, an AAVrh10-based gene therapy candidate that is designed to deliver functional *FXN* intravenously for the treatment of FA cardiomyopathy, 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 Friedreich's ataxia. The FDA has granted Rare Pediatric Disease designation and Orphan Drug designation to LX2006 for the treatment of Friedreich's ataxia. Our IND for LX2006 was cleared by the FDA in 2022, and we expect to initiate an open-label, dose-escalation Phase 1/2 clinical trial in patients with FA cardiomyopathy in mid-2022, with interim biomarker data expected by the end of 2022.

Overview of Friedreich's ataxia

Friedreich's ataxia is a genetic, progressive, degenerative multi-system disorder with a prevalence of 1:40,000 or approximately 8,000 people in the United States. It is estimated that approximately 70% or 5,600 of these patients will develop FA cardiomyopathy. In the European Union, Friedreich's ataxia affects approximately 11,000 people, with approximately 8,000 of these cases projected to include FA cardiomyopathy. Friedreich's ataxia 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. Friedreich's ataxia patients with classically presenting disease have peripheral, non-cardiac tissue frataxin protein levels that range from 2% to 30% of normal levels. Carriers who do not develop symptoms of Friedreich's ataxia generally have peripheral tissue frataxin protein levels that range from 30% to 80% of normal levels. This frataxin deficiency in the mitochondria of myocardial cells causes disruption of normal iron-sulfur cluster biosynthesis leading to mitochondrial dysfunction and deficient energy production, as shown below.

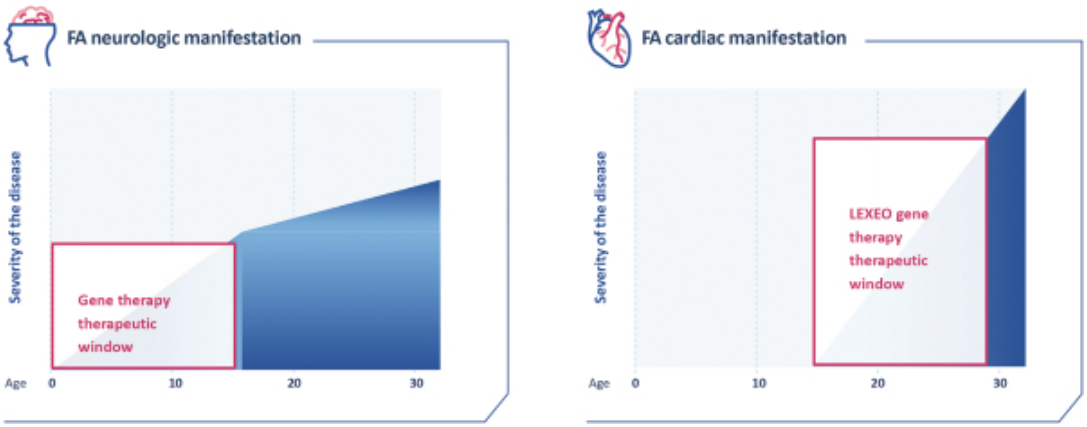
Overview of FA cardiomyopathy disease mechanism



The neurologic disease and cardiac disease are two distinct manifestations of Friedreich's ataxia. 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 muscle, or hypertrophic cardiomyopathy, and arrhythmias. Hypertrophic cardiomyopathy, fibrosis, heart failure and arrhythmias are the cause of death in approximately two-thirds of Friedreich's ataxia patients. Typical onset of the cardiac disease is 15 to 30 years old.

By the time the cardiac disease of Friedreich's ataxia emerges, the neurologic disease is generally significantly advanced and may not be amenable to gene therapy. The optimal patient age for treatment of the neurologic disease is between five and 15 years of age while the optimal patient age for demonstrating treatment effect of the cardiac disease is 15 to 30 years of age. There are currently no approved treatments for either the neurological or cardiac manifestations of Friedreich's ataxia. As a result, patients with Friedreich's ataxia have significant unmet need.

Neurological disease and cardiac disease are distinct manifestations of Friedreich's ataxia with different potential therapeutic treatment windows

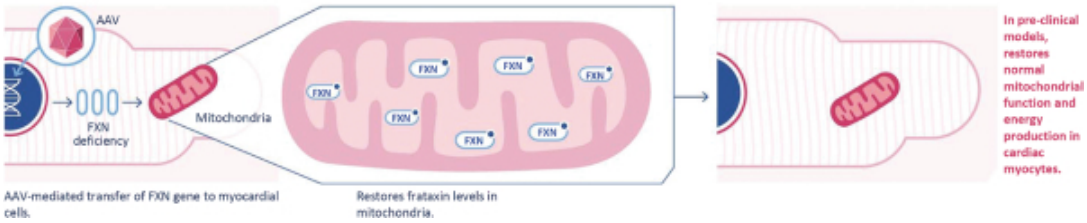


Our solution: LX2006

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 promotor 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. In addition, there is a lower rate of pre-existing antibodies with AAVrh10 when compared to other commonly used vectors, such as AAV9 and AAV2.

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 restores 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

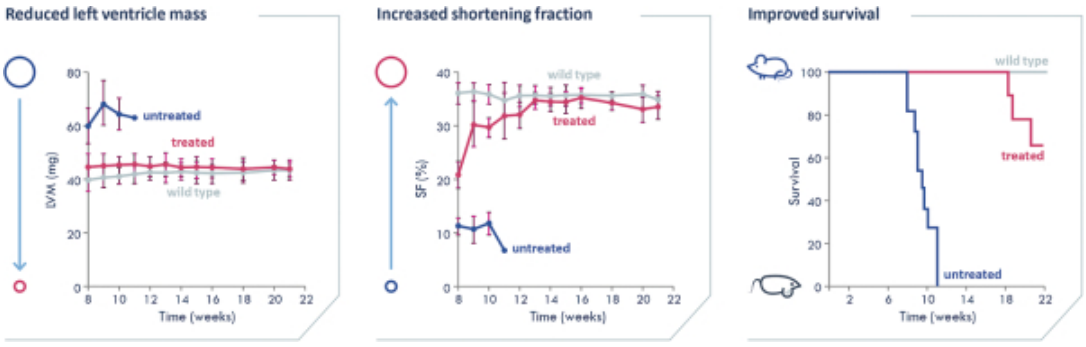


Preclinical studies in Friedreich's ataxia mouse models

LX2006 has demonstrated the ability to significantly reverse the cardiac phenotype in preclinical studies. The ability to restore FXN expression levels using an AAVrh10 vector has been established in numerous preclinical studies of Friedreich's ataxia conducted internally and by third parties. The most frequently used preclinical mouse models for Friedreich's ataxia are the MCK model which represents the most severe phenotype of FA cardiomyopathy, and the Myosin heavy chain, aisoform, or aMyHC, model, which represents a milder phenotype.

In the preclinical studies conducted by Weill Cornell Medicine utilizing the MCK model, LX2006 reduced left ventricle mass, increased shortening fraction, or pumping ability of the heart, and increased survival, as shown in the graphic below. The MCK model completely lacks the myocardial FXN protein and untreated mice have a lifespan of approximately 11 weeks of age due to heart failure. The untreated mouse cohort developed left ventricular hypertrophy starting at four to five weeks of age, which is associated with a rapid and progressive geometric remodeling of the heart. This cohort exhibited decreased systolic function at eight weeks of age, which led to a severe decrease in resting cardiac output. When administered to the MCK model at three weeks, LX2006 prevented the onset of the disease. In addition, when administered at seven weeks, LX2006 reduced hypertrophy and normalized heart function, based on shortening fraction, within five weeks, and improved survival, as shown in the graphic below. Based on these results, we believe LX2006 has the potential to improve cardiac function and reverse the disease abnormalities in FA cardiomyopathy patients.

LX2006 improves cardiac function and survival in severe *FXN* knockout model

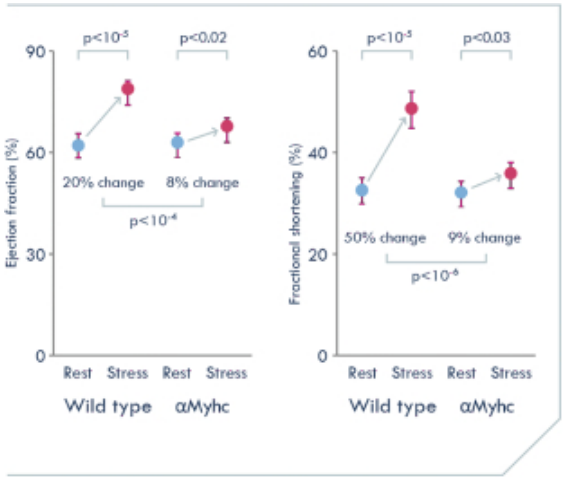


Wild type mice, n=10; untreated MCK mice, n=9; and treated MCK mice, n=9. The treated MCK mice were given a dose of 5.4 x10^{13} vg/kg at seven weeks of age.

Additionally, we have completed preclinical studies of LX2006 using a aMyHC mouse model. The aMyHC model has myocardial frataxin protein levels that are less than 50% of normal frataxin expression levels and present with a milder cardiac phenotype. When the mouse is given dobutamine to induce cardiac stress, the cardiac disease becomes apparent, similar to the mild phenotype in FA cardiomyopathy patients at an early stage of their cardiac disease.

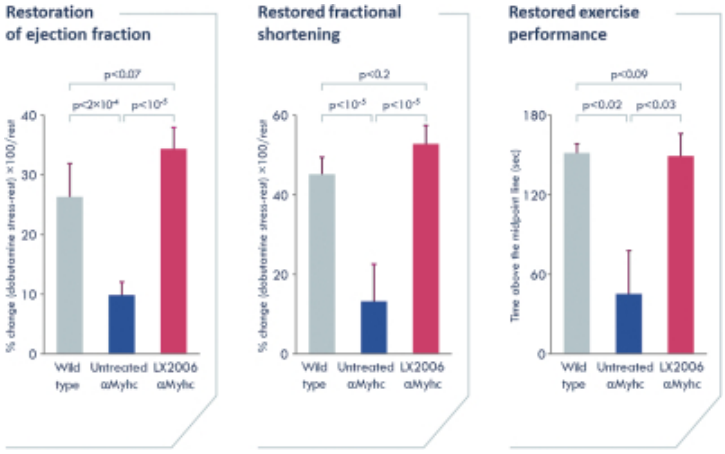
The figures below show that aMyHC mouse models display ejection fraction and fractional shortening levels similar to the wild type phenotype when at rest. However, when both wild type and aMyHC animals are stressed by the administration of dobutamine, the model demonstrated that ejection fraction and fractional shortening levels for the aMyHC mice were below wild type levels.

aMyhc mouse model has diminished cardiac function in response to induced stress



In the aMyHC mouse model, as demonstrated in the graphic below, intravenous administration of LX2006 at a dose of 4 x10^{12} vg/kg resulted in improvements in the cardiac phenotype as indicated by correction of dobutamine stress-induced ejection fraction and fractional shortening as well as improvement in treadmill performance. These results show that LX2006 improves key cardiac function measures to wild type levels, helping reverse the pathology of FA cardiomyopathy and suggesting that it could potentially improve cardiac function in Friedreich's ataxia patients.

LX2006 reverses aMyHC cardiac phenotype in key cardiac functional measures in preclinical models



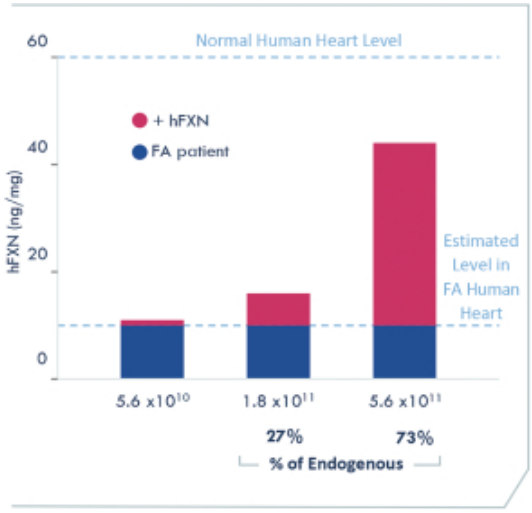
Preclinical dose range finding study

Differences between Friedreich's ataxia patients and phenotypically normal carriers of Friedreich's ataxia mutations suggest that achieving approximately 30% of normal endogenous frataxin levels would have a potential clinical beneficial effect in Friedreich's ataxia patients.

Seven-week-old MCK mice were administered 5.6 x10^{10} vg/kg, 1.8 x10^{11} vg/kg and 5.6 x10^{12} vg/kg of LX2006 and evaluated for efficacy as a measure of body weight improvement, survival and cardiac function, and associated myocardial FXN expression at one month post-dose. MCK mice showed a dose-dependent improvement in efficacy with LX2006. In these mice the dose of 1.8 x10^{11} vg/kg resulted in trends of improvement in body weight and cardiac function survival, and the level of myocardial frataxin was 27% of normal human levels. The dose of 5.6 x10^{11} vg/kg resulted in statistically significant improvements in body weight, cardiac function, and survival, and the level of myocardial frataxin expression was 73% of normal human levels.

To establish the endogenous levels in the myocardial cells of non-Friedreich's ataxia subjects, cardiac samples were obtained during autopsy from five different individuals who did not have Friedreich's ataxia. The average level of myocardial frataxin measured in non-Friedreich's ataxia patient samples was approximately 60 ng/mg protein (represented by the upper light blue dashed line in the figure below). In Friedreich's ataxia patients, an average of 16% of normal endogenous levels would lead to an estimate of 10 ng/mg protein in myocardial cells (represented by the lower light blue dashed line and the blue bars in the figure below).

Dose dependent FXN expression in MCK mouse model

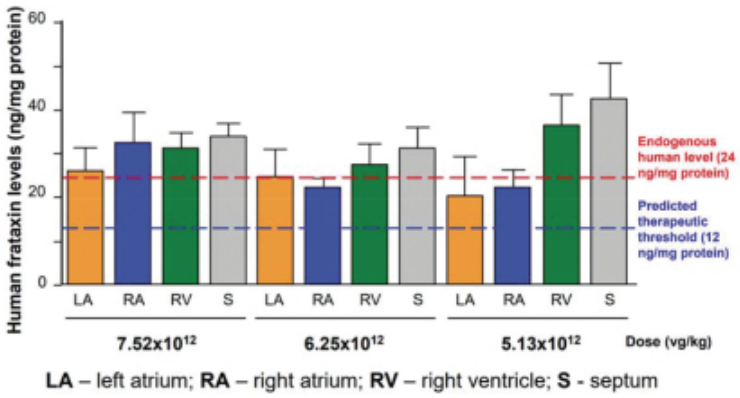


Since the myocardial FXN expression in response to LX2006 treatment in a Friedreich's ataxia patient's stressed heart is expected to be similar to the high level of expression in MCK mice observed upon administration of LX2006, dose selection for first-in-human study was based on the MCK mouse data. The 1.8 x10^{11} vg/kg dose, which showed 27% of normal myocardial FXN expression, was identified as the minimally effective dose, and the 5.6 x10^{11} vg/kg dose, which showed 73% of myocardial FXN expression, was identified as the significantly effective dose.

Preclinical expression study

In a separate NHP study conducted at a third party lab in cynomolgus monkeys that were administered doses ranging from 5.13 x10^{12} to 7.52 x10^{12} vg/kg of LX2006, the frataxin expression in myocardial tissue at three weeks following administration exceeded the predicted therapeutic threshold and, in some cases, higher than the endogenous FXN level in humans, in the atrium, septum and right ventricle, as shown in the figure below. This data was presented at the 2021 American Society of Gene and Cell Therapy.

LX2006 expresses frataxin levels in NHP Cardiac Tissues that exceed predicted therapeutic levels



Preclinical safety studies

In a safety study of African green monkeys, LX2006 doses of 1.2 x10^{12} vg/kg and 3.9 x10^{12} vg/kg resulted in dose-dependent increases in myocardial frataxin expression three months post dose. The dose of 1.2 x10^{12} vg/kg resulted in FXN expression which is 22% of normal human FXN, or hFXN, levels; while the dose of 3.9 x10^{12} vg/kg resulted in FXN expression which is 45% of normal hFXN levels. Both doses demonstrated favorable safety profiles, including no effects on cardiac pathology, physiology and echocardiography. The liver showed minimal to moderate vacuoles, or membrane-enclosed spaces in cells, not considered clinically significant, with no evidence of liver injury. No dorsal root ganglia toxicity was observed.

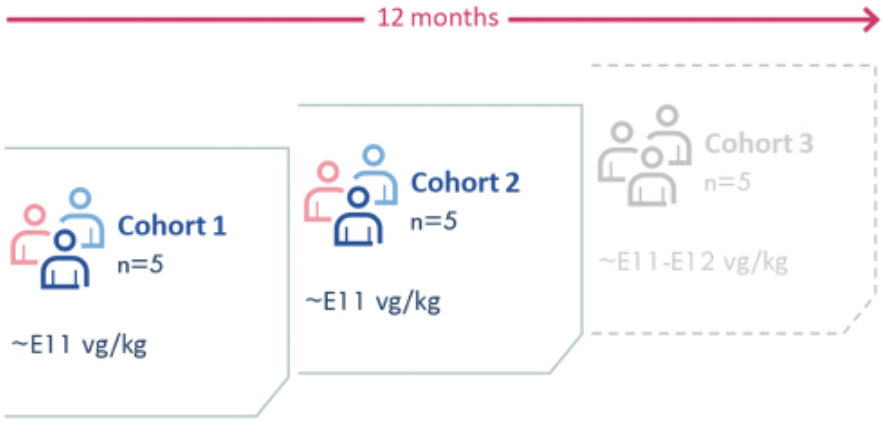
The safety of LX2006 was also tested in seven-week-old wild type mice administered 1.2 x10^{12}, 3.7 x10^{12}, and 1.2 x10^{13} vg/kg for one and three months, and 5.6 x10^{11}, 1.8 x10^{12}, and 5.6 x10^{12} vg/kg for 10 months. Animals in the one- and three-month cohorts survived to their scheduled terminations, while those in the 10-month cohort had body weight loss at two time periods that necessitated early euthanasia. The first timepoint was approximately six weeks for four of 12 males in the 5.6 x10^{12} vg/kg 10-month cohort; the weight loss was attributed to liver toxicity, which may have been related to high levels of liver hFXN, although hFXN levels were not determined in these mice. No adverse effects occurred in females up to three months post-dose. At 30 weeks post-dose, the remaining animals in the high dose (5.6 x10^{12} vg/kg) group and six of 12 female mice in the mid dose (1.8 x10^{12} vg/kg) group had minimal to moderate hepatic changes and weight loss necessitating early euthanasia. It is possible that both hepatic and possibly cardiac frataxin levels may have contributed to the weight loss. No dorsal root ganglia toxicity was observed at any doses in any animals.

The dose of 5.6 x10^{11} vg/kg in the 10-month cohort was generally well tolerated with no treatment-related clinical signs, body weight changes, hematologic or clinical chemistry findings, nor histopathology findings, except for hepatocellular carcinoma, or HCC, which was observed in one of six males at 5.6 x10^{11} vg/kg and in three of six males at 1.8 x10^{12} vg/kg. While this finding was considered adverse in the context of the study, a large body of available data suggests that HCC observed in mice after AAV treatment is unlikely to translate to risks for humans, as it has not been observed in higher species or humans (FDA 2021). The incidence of HCC has been seen in multiple long-term mouse studies with systemic administration of other AAVs.

LX2006 clinical development and trial design

Our IND for LX2006 was cleared by the FDA in 2022, and we expect to initiate a Phase 1/2 clinical trial of LX2006 in patients with FA cardiomyopathy in mid-2022. The Phase 1/2 study is a first-in-human, 52-week, dose-ascending, open-label trial of LX2006 in patients who have FA cardiomyopathy. LX2006 will be administered as a one-time intravenous infusion to patients in at least two dose-ascending cohorts. The trial will consist of five patients in each cohort. A potential third cohort will receive a dose that will be selected based on the dose-response relationships seen in cohort 1 and cohort 2 and an additional NHP study that we are conducting concurrently with the first two dose cohorts in our clinical trial. 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.

Design of phase 1/2 clinical trial of LX2006



Key patient inclusion criteria for the study include genotyping, abnormal cardiopulmonary exercise testing, or CPET, peak oxygen consumption, or peak VO2, levels, left ventricular hypertrophy, abnormal cardiac strain, ejection fraction ≥45%, and focal fibrosis ≤5% of cardiac wall mass. The primary endpoint of the Phase 1/2 trial is to assess the safety and tolerability of one-time administration of LX2006 for the purpose of selecting the appropriate dose for further clinical development. The secondary 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.
- *Cardiac MRI*: All patients undergo MRI scans of the heart to measure left ventricular mass index, left ventricular ejection fraction, stroke volume, strain, and fibrosis.
- *Cardiac Echo*: All patients undergo cardiac ECHO to measure left ventricular mass index, left ventricular ejection fraction and strain.
- *Cardiac biopsy*: Biopsies will measure, among other things, frataxin protein expression.
- *Biomarkers*: Serum cardiac biomarkers, including troponin.

LX2020 for the treatment of ARVC caused by PKP2 *mutations*

We are developing LX2020 as an AAVrh10-based gene therapy for the treatment of ARVC caused by mutations in the *PKP2* gene. LX2020 is designed to provide a fully functional *PKP2* gene to increase desmosomal PKP2

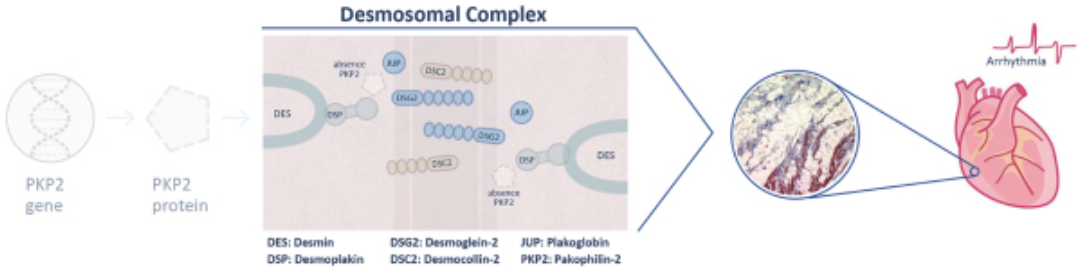
protein levels, reassemble desmosomes, and prevent cardiac arrhythmias and prevent or treat cardiac dysfunction. *PKP2* is the most frequently mutated desmosomal gene and we believe there are more than 70,000 ARVC patients affected by the *PKP2* mutation in the United States. We intend to submit an IND to the FDA for LX2020 by the end of 2023.

Overview of ARVC

ARVC is a genetic heart disease primarily characterized by myocardial cell loss and the replacement of heart muscle with fibrotic tissue and fatty deposits. ARVC 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. The disease is associated with high mortality as more than 40% of ARVC patients die or have heart transplantation within 10 years of diagnosis.

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

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



We believe that ARVC has an estimated prevalence in the general US population between 1:2000 and 1:5000 and estimate that over half of all ARVC patients have a genetic form of the disease. Five desmosomal genes account for nearly all genetic cases of ARVC. We are targeting the *PKP2* gene because mutations in this gene are the most common known genetic cause of ARVC. No effective treatments or cures for ARVC exist, and thus, strategies targeted at elevating PKP2 protein levels represent a clinically relevant avenue to treat a large portion of ARVC populations. We believe that mutations in the *PKP2* gene are associated with approximately 75% of all genetic cases of ARVC, resulting in approximately 70,000 and 95,000 ARVC 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.

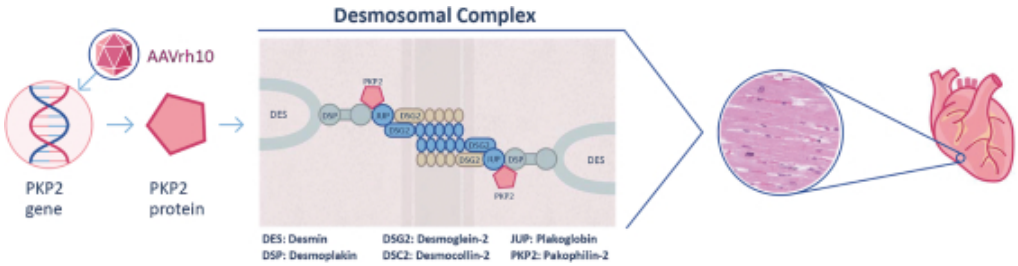
Symptoms of the disease can include palpitations, dizziness, heart failure and sudden death. Abnormal function of the right ventricle, fatty or fibrotic infiltrates in the myocardium, abnormal ECG, arrhythmias, or a family history of ARVC can all lead physicians to diagnose the disease.

Our solution: LX2020

We are developing LX2020 as an AAVrh10-based gene therapy candidate for the treatment of ARVC caused by mutations in the *PKP2* gene. LX2020 is designed to intravenously deliver a fully functional *PKP2* gene to cardiac muscle to increase desmosomal PKP2 protein levels and restore myocardial cell function. We believe that by delivering a fully functional *PKP2* gene, LX2020 has the potential to address the underlying cause of ARVC

for many patients, by reassembling the cardiac desmosomes, preventing cardiac arrhythmias and preventing or treating cardiac dysfunction and having a significant effect on lifespan.

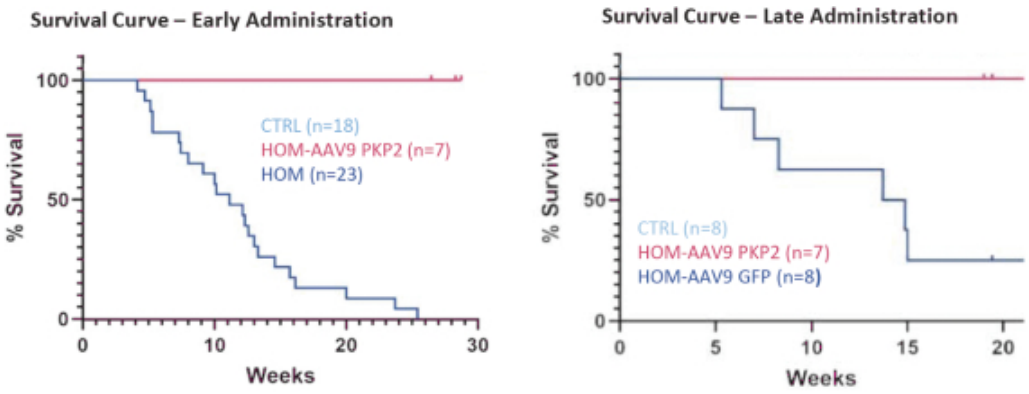
LX2020 is designed to deliver functional *PKP2* to restore normal desmosomal function



Preclinical studies

Our preclinical studies have demonstrated that delivery of *PKP2* led to reassembling of the cardiac desmosome, as well as prevented arrhythmias and cardiac dysfunction, and increased survival in a genetic mouse model of ARVC harboring *PKP2* patient genetics. The graphs below show survival data for the mice following administration of an AAV9-based *PKP2* transgene at a dose of 5 x10^{11} vg/mouse. This was done both before severe disease onset on day two and after severe disease onset at four weeks. With early administration, we observed that 100% of the treated mice were alive at 30 weeks, while the untreated *PKP2*-homozygous mutant mice all died by approximately 25 weeks. With late administration, we observed that 100% of the treated mice were alive at 20 weeks, while only 20% of the green fluorescent protein-treated *PKP2*-homozygous mutant mice were alive at 20 weeks at the time data were presented from the study.

Preclinical data of early and late stage administration of AAV9-*PKP2* improved survival in ARVC mouse model harboring *PKP2* patient genetics



Based on these results, we believe that the administration of AAV-*PKP2* has the potential to restore the cardiac desmosomes and treat ARVC caused by a mutation in this gene. We further believe that early administration has the potential to prevent disease onset, improve cardiac symptoms, and prolong survival, while late administration may also have benefits in terms of restoring desmosomes, improving cardiac function and prolonging life.

The preclinical findings to date provide direction for our ongoing development efforts. We plan to conduct further preclinical studies using an optimized AAVrh10-*PKP2* transgene in several murine models to generate additional data to better inform our clinical development plans.

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: We are designing LX2021 to treat adults with ARVC irrespective of the underlying genetic mutation by delivering the coding sequence for functional Cx43 protein. We believe that restoring the Cx43 protein has the potential to treat multiple genetic causes of ARVC because cardiac loss of Cx43 is a molecular deficit that is generally observed in all ARVC populations studied. Gap junctions facilitate electrical coupling between myocardial cells. These structures are formed by connexins, and Cx43 is the protein constitutively expressed at the highest level. In patients with heart disease, including heart failure, Cx43 can be relocalized in the lateral walls of myocardial cells and is significantly reduced at cardiac muscle cell junctions, especially in ARVC populations. Preclinical data from a desmoplankin loss-of-function murine model, which exhibits the most severe version of ARVC, demonstrate that severely diseased ARVC mice treated with AAV-based therapy expressing Cx43, can reassemble the cardiac cell junction and desmosome as well as display significantly fewer arrhythmias, have improved cardiac function and have almost twice the survival duration as untreated adult mice. The preclinical studies and findings to date provide direction for our ongoing development efforts. We plan to initiate IND-enabling studies for LX2021 by the end of 2023.

LX2022: We are designing LX2022 to treat HCM due to mutations in the *TNNI3* gene by delivering a functional *TNNI3* gene to myocardial cells. With an estimated prevalence of 1:500, HCM is one of the most common forms of genetic cardiomyopathy and is caused by mutations that affect the cardiac sarcomere in approximately 75% of cases. It is inherited as an autosomal dominant trait, with over 500,000 patients in the United States alone who have a genetic form of HCM. It is estimated that approximately 30,000 patients in the United States have HCM due to mutations in the thin-filament gene *TNNI3*. In the European Union, we believe there are approximately 36,000 patients with TNNI3-associated HCM. The *TNNI3* gene encodes troponin I, a key protein in the thin filament of the sarcomere, which is involved in cardiac contraction and relaxation. Mutations in the gene result in left ventricular hypertrophy and dysfunction, which can lead to heart failure, and increased risk of arrhythmias. Our preclinical studies and findings to date provide direction for our ongoing development efforts. We plan to initiate IND-enabling studies for LX2022 by the end of 2024.

Our CNS gene therapy programs

Our Alzheimer’s disease franchise

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 believe LX1001 is the only clinical-stage gene therapy candidate designed to treat this genetic cause 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-population and can help inform the development plan of our next generation candidates LX1020 and LX1021 which we believe could demonstrate an even more dramatic effect to slow or halt the progression 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 the ongoing Phase 1/2 clinical trial of LX1001, two patients from the first dose cohort have demonstrated decline in CSF tau biomarkers over 12 months. We have also reported data demonstrating expression of the protective APOE2 protein in all four patients in the low-dose cohort with follow-up data.
- *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.
- *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.

LX1001 for the treatment of homozygous APOE4-associated Alzheimer's disease

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-population we believe will be the most likely to demonstrate a potential treatment effect from *APOE2*-based gene therapy. We anticipate reporting biomarker data from the mid-dose cohort by the end of 2022.

Overview on Alzheimer's disease

Background of Alzheimer's disease

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 with Alzheimer's disease in 2021 in the United States alone, 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.

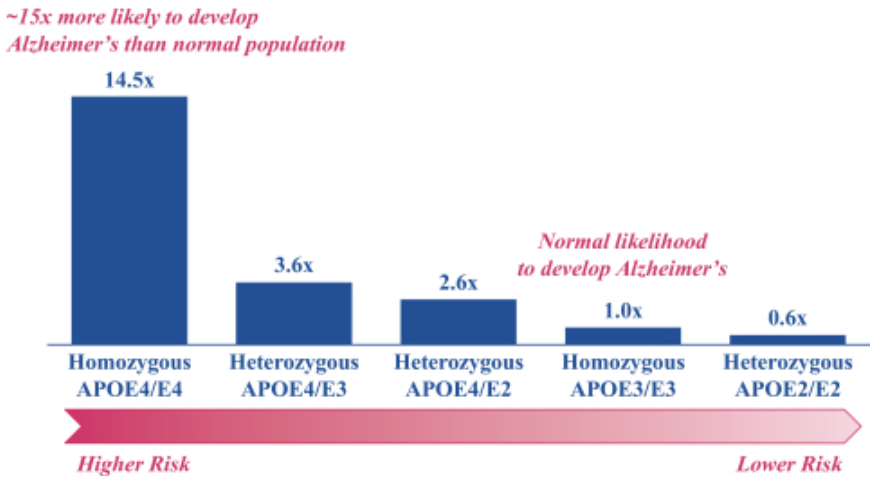
Alzheimer's disease is characterized by a complex underlying pathology in the CNS, including accumulation of Aß plaques, abnormal phosphorylation of tau, development of tau tangles, inflammation, and progressive loss of neurons, all of which combine to precipitate a progressive decline in cognitive function. 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 current standard of care for Alzheimer's disease is symptomatic therapies, such as donepezil (Aricept) and memantine (Namenda), that are viewed to have limited benefit and do not address the underlying cause of the disease. Recently, the FDA granted accelerated approval to Biogen, Inc. for the anti-amyloid antibody intravenous infusion therapy aducanumab (Aduhelm), for the treatment of patients with mild dementia or mild cognitive impairment stages of Alzheimer's disease. This represents a potential approval pathway for future 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)



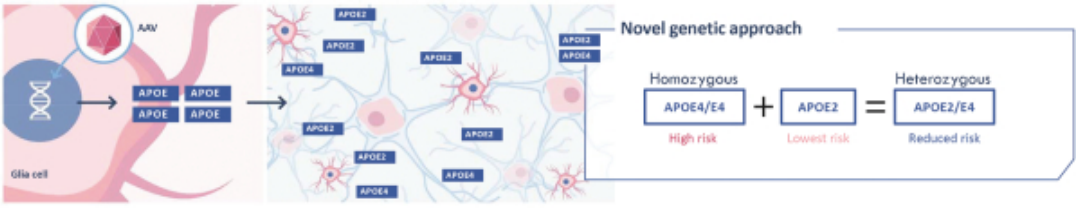
Third-party genetic epidemiology data from humans suggest that *APOE4* and *APOE2* are co-dominant, or both expressed, such that *APOE2/E4* heterozygous individuals have a substantially reduced risk of developing the disease as compared to *APOE4/E4* homozygous individuals. Therefore, the expression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

In light of the limited success in developing therapies that effectively address Aß, tau and other molecules involved in Alzheimer's disease pathology, 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 solution: LX1001

LX1001 is an AAVrh10-based gene therapy designed to deliver the human *APOE2* gene into the CNS via one-time administration to the CSF, for the treatment of *APOE4* homozygous patients. LX1001 is designed to express the protective APOE2 in the CNS 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.

Preclinical studies

Weill Cornell Medicine has conducted preclinical studies in large and small animals that have demonstrated the potential for LX1001 to induce APOE2 expression levels that we believe will be sufficient to halt or slow the progression of Alzheimer's disease in *APOE4* homozygous patients.

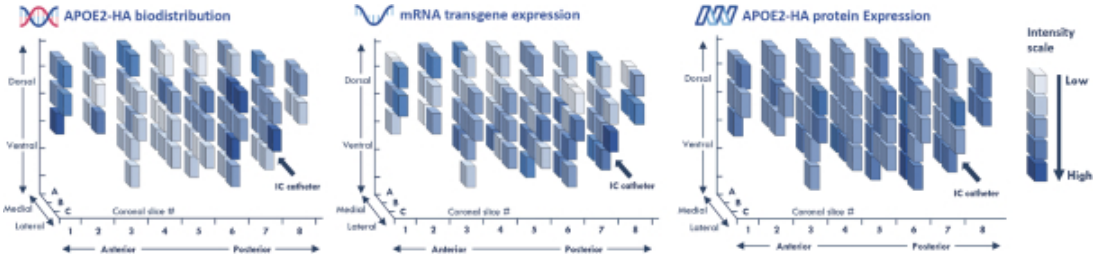
Preclinical murine studies of LX1001 were conducted in a widely used model of brain amyloidosis (PDAPP mice) as well as a mouse model that develops APOE4-dependent amyloid deposition (APP.PS1/TRE4 mice). APP.PS1/TRE4 triple transgenic mice develop early and robust Aß and amyloid deposition, which is dramatically reduced in the absence of APOE4. Intracerebral delivery of LX1001 led to the widespread brain expression of APOE2. Studies in the APP.PS1/TRE4 model showed an approximately 80% reduction in insoluble Aß1-42 in the hippocampus eight weeks following intrahippocampal administration of LX1001.

An additional preclinical study in NHPs was conducted to determine the most active and well-tolerated route of administration for LX1001 to achieve broad CNS biodistribution and therapeutic levels of the APOE2 proteins. In these studies, we utilized an intracisternal delivery route to deliver the gene therapy vector to the CSF in order to achieve widespread biodistribution of LX1001. The intracisternal route has been shown in multiple studies in large animals to result in transgene expression throughout the CNS and involves a minimally invasive procedure to infuse into the CSF.

To evaluate this route of delivery, at eight weeks following intracisternal administration of total vector dose of 5 x10^{13} vg/ml CSF of LX1001, with the *APOE* transgene tagged with the common antibody epitope tag hemagglutinin, or APOE-HA, three NHP brains in one study were evaluated for the extent of vector APOE2-HA biodistribution, mRNA transgene expression, and APOE2-HA protein expression. To visualize the extent of vector biodistribution and transgene and protein expression, a 3D representation of one centimeter cubes of the brain was used, as shown below in the example of one NHP. As expected for a secreted protein like APOE2, diffuse expression was observed throughout the brain. Greater-than-baseline levels of all three targets were

observed in most cubes throughout the CNS, as well as in the hippocampal regions, the area of the brain most relevant in Alzheimer's disease.

Biodistribution of APOE2-HA vector genomic copies, transgene mRNA, and protein levels in brain of an NHP following intracisternal administration of LX1001



The results from this study demonstrate that *LX1001* was consistently expressed through the brain in a NHPs and achieved broad distribution in the CNS of the animals. In addition, administration of the HA tagged version of LX1001 directly to the CNS of NHPs exhibited a favorable safety profile.

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 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 a maximum tolerable 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 Aß42, 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 three ascending dose cohorts (5.0 x10^{10}, 1.6 x10^{11} and 5.0 x10^{11} vg/ml), with the dose for each patient determined based on CSF volume measured by MRI. Each dose cohort will consist of approximately five patients for a total of approximately 15 patients in the entire trial. Key patient enrollment criteria include *APOE4* homozygous genetic profile, patient age of 50 years or older, positive amyloid PET scan, 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.

Design of phase 1/2 clinical trial of LX1001

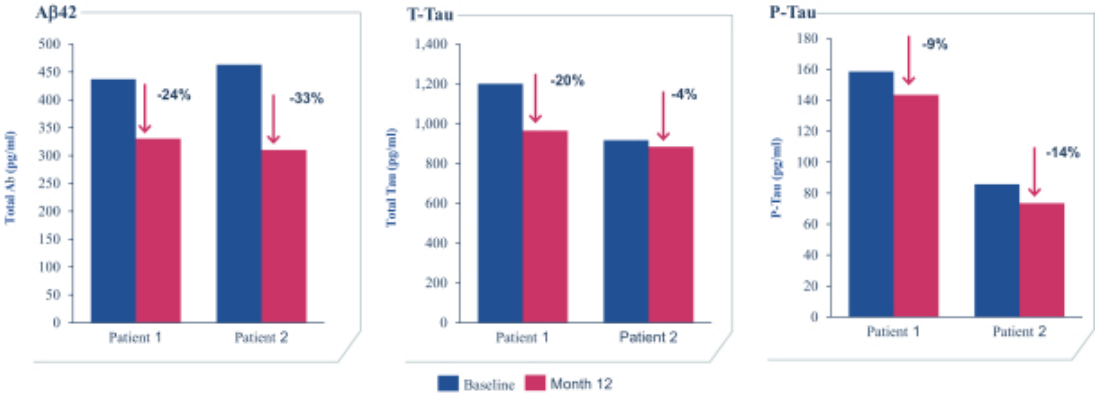
Trial results

Preliminary data for cohort 1, which is the low-dose group, showed increases in CSF APOE2 levels, from baseline levels of 0, relative to their respective CSF APOE4 levels, in all four patients with follow up data at three months or longer. These results, shown below, demonstrate that the *APOE2* transgene is being expressed in the CNS in all four of these patients.

Interim CSF APOE2/APOE4 expression data from cohort 1

The two patients for whom we have 12-month data in cohort 1, both of whom had moderate Alzheimer's disease at baseline, showed declines in CSF core biomarkers Aß42, t-tau and p-tau over 12 months, as shown below.

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



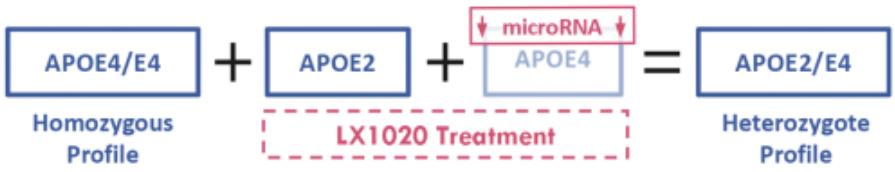
Among all patients in cohort 1, treatment with LX1001 has been well-tolerated with no serious adverse events reported to date. Overall, initial preliminary data in the low-dose cohort suggest that LX1001 has promise as a therapeutic for *APOE4* homozygous Alzheimer's disease patients. We expect to report the initial biomarker data from the mid-dose cohort by the end of 2022.

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

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

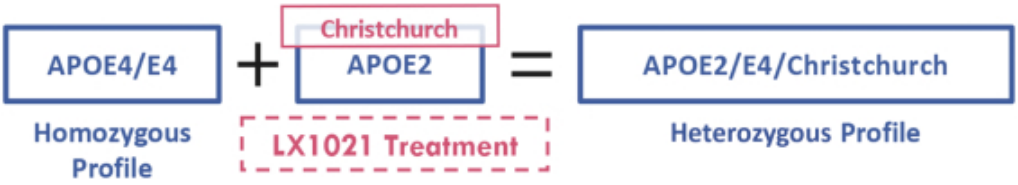
LX1020: We are designing LX1020 to treat *APOE4* homozygous patients by delivering both the protective *APOE2* allele and miRNA to suppress *APOE4*. We believe delivery of *APOE2* with concurrent suppression of *APOE4* will achieve a higher degree of conversion to the *APOE4/E2* heterozygous profile, which should lead to greater therapeutic effect. We plan to initiate IND-enabling studies for LX1020 by the end of 2023.

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



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, molecules found on the surface of neurons, which 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 initiate IND enabling studies for LX1021 by the end of 2024.

LX2021 is designed to add a christchurch mutation-modified *APOE2* gene to the CNS of *APOE4* homozygous patients



LX1004 for the treatment of CLN2 Batten disease

We are developing LX1004, an AAVrh10-based gene therapy candidate designed to deliver a fully functional *CLN2* gene to the CNS via intracisternal injection in order to restore TPP1, the secreted protein that is deficient in CLN2 Batten disease. The FDA has granted Rare Pediatric Disease designation and Orphan Drug designation to LX1004 for the treatment of CLN2 disease. In a Phase 1/2 trial that was conducted by Weill Cornell Medicine under an IND that was subsequently transferred to us, LX1004 demonstrated an increase in TPP1 levels leading to a slower decline of motor and language function in treated patients compared to natural history controls, at 18 months post treatment. We intend to initiate a pivotal Phase 2/3 trial of LX1004 in the first half of 2023.

Overview of Batten disease

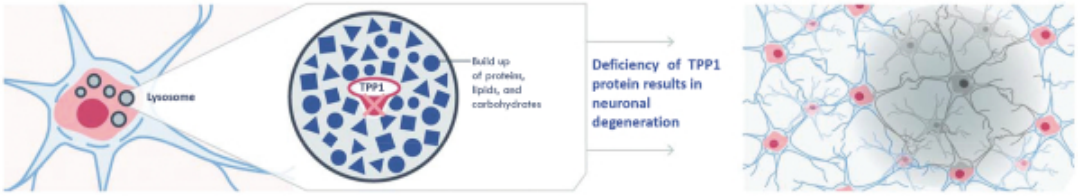
Batten disease is the common name for a broad class of rare, fatal, inherited disorders of the nervous system also known as neuronal ceroid lipofuscinoses. In each form of Batten disease a defect in a specific gene interferes

with a particular neuronal function. Each defective gene is given a different number designation (ranging from *CLN1-8* and *CLN10-14* for the 13 known forms of Batten disease). The different designations have generally similar clinical features but vary in severity and the age of onset.

CLN2 Batten disease is caused by a defect in the *CLN2* gene found on chromosome 11 and affects up to approximately 900 patients in the United States and European Union. The *CLN2* gene produces TPP1, which breaks down proteins in the lysosomes of neurons. In the case of CLN2 Batten disease, there is enzyme deficiency, resulting in neuronal degeneration from build-up of non-degraded proteins. Developmental delay begins around the end of age two and children progressively lose motor and cognitive function, become unable to communicate and develop seizures and blindness. Most children with CLN2 Batten disease die between the ages of six and twelve.

There is no cure for the disorder. Cerliponase alfa (Brineura), an enzyme replacement therapy, is the only FDA approved treatment for CLN2 Batten disease. Brineura requires chronic twice monthly infusions directly into the CSF via intraventricular injections. Treatment also requires that an intraventricular access device be maintained chronically, and which has been associated with infections and other complications.

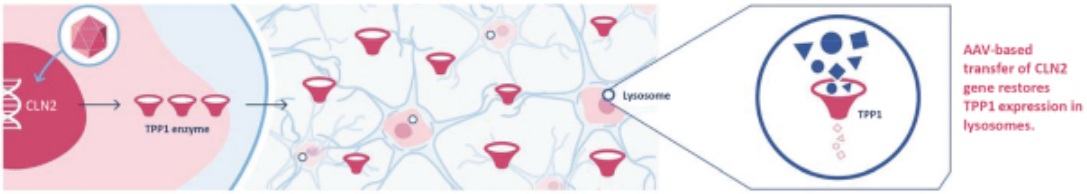
Overview of CLN2 Batten disease mechanism



Our solution: LX1004

We are developing LX1004, an AAVrh10-based gene therapy candidate designed to deliver a fully-functional *CLN2* gene, to restore TPP1 expression in neuronal lysosomes, in order to have a neuroprotective effect in CLN2 Batten disease, as illustrated below. TPP1 is a secreted protein and capable of cross-correcting neighboring cells with uptake mediated via the mannose-6-receptor pathway. We believe LX1004 has the potential to address the underlying cause of the disorder.

LX1004 is designed to deliver functional TPP1 and restored TPP1 expression in lysosomes

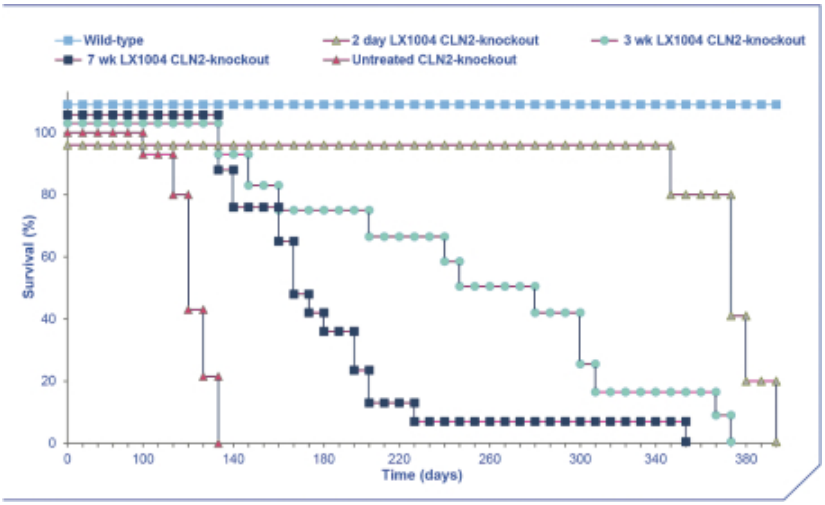


Preclinical study

A preclinical study conducted by us demonstrated that administration of LX1004 conferred a statistically significant survival benefit in *CLN2*-knockout mice, as shown in the graphic below. The conventional method for measuring the statistical significance of a result is known as the “p-value,” which represents the probability that random chance caused the result (e.g., a p-value = 0.001 means that there is a 0.1% or less probability that the difference between the control group and the treatment group is purely due to random chance). Generally, a p-value less than 0.05 is considered statistically significant, and may be supportive of a finding of efficacy by regulatory authorities.

Median ages of survival of the mice that were treated at ages seven weeks, three weeks, and two days were 168 days, 277 days, and 376 days, respectively, compared to a median age of survival of 121 days for untreated *CLN2*-knockout mice. Kaplan-Meier analysis, which is a statistical method to analyze survival data, revealed a statistically significant survival advantage for the mice treated at three weeks of age over the mice treated at seven weeks of age ($p<0.001$), and the survival of the mice treated at two days of age was significantly longer than both the mice treated at seven weeks of age ($p<0.0001$) and the mice treated at three weeks of age ($p<0.01$). This study demonstrated that the mice treated with LX1004 at the earliest age showed the most improved median age of survival.

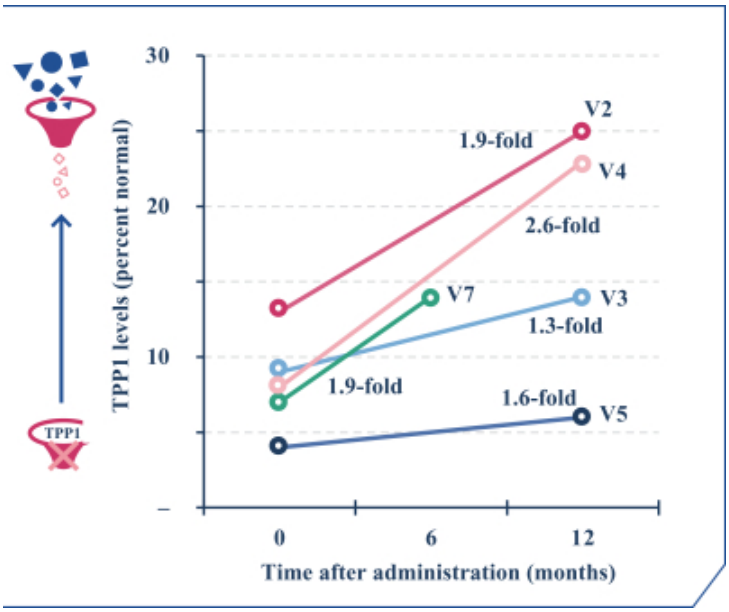
Preclinical study shows survival benefit of LX1004 in treated *CLN2*-knockout Mice



Phase 1/2 clinical trial results

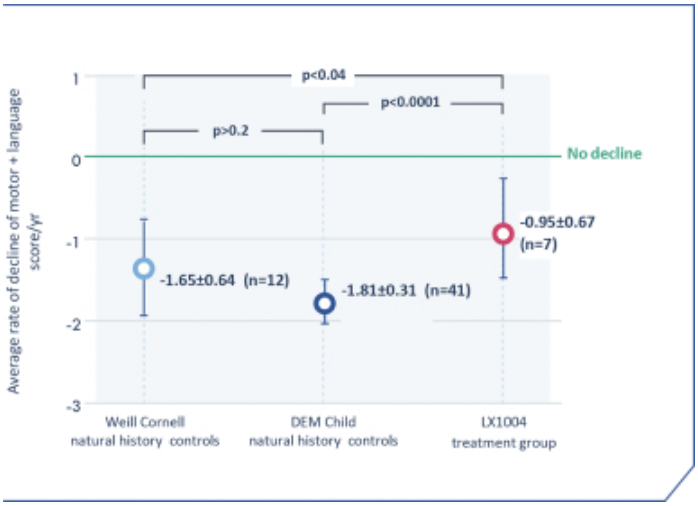
In a Phase 1/2 clinical trial conducted by Weill Cornell Medicine, LX1004 was administered intraparenchymally to the CNS of eight children with CLN2 Batten disease. Seven children had follow-up visits during the 18-month duration of the study, and of these, five children had CSF samples pre- and post-treatment. All five of those patients had increased TPP1 levels in their CSF up to 12 months, indicating substantial expression of the protein and durability of the therapy.

LX1004 demonstrated increases in TPP1 expression levels up to 12 months



Additionally, as demonstrated in the below graphic, the treated patients had a statistically significant reduction in the rate of disease progression compared to natural history controls. The trial used the established CLN2 Clinical Rating Scale to assess efficacy. This scale was developed to assess the loss of function in CLN2 Batten disease patients that occurs over the course of the disease. It is a four-part assessment of language, motor function, visual function, and seizures. The rate of decline in the motor + language subscores of the treatment group was compared to two historical control groups of untreated patients: the Weill Cornell Medicine natural history cohort and the European DEM Child natural history cohort. The annual rate of decline was similar in the two control groups. In contrast, the rate of decline in the treated patients was slower than that in both control groups.

LX1004 demonstrated a slower rate of decline relative to natural history controls



Furthermore, the data demonstrated that LX1004 was well tolerated, with minimal serious adverse events, and findings on brain MRI (hyperintensities at the site of the catheter tip, where the highest concentration of vector occurred) that were not considered clinically meaningful.

Future development

Based on results of the Phase 1/2 clinical trial and preclinical studies, we believe that improved dosing and biodistribution could result in even greater clinical benefit in slowing or halting disease progression, in the Phase 2/3 trial compared to the Phase 1/2 trial. We plan to pursue approaches that could improve the outcome of treatment in the potentially pivotal trial that we are currently designing. We expect to change the route of administration from intraparenchymal to intracisternal delivery, which has demonstrated better biodistribution of transgenes and will allow for significantly higher doses. To help guide future development plans, we are conducting additional NHP studies of LX1004 administered via intracisternal injection to assess the biodistribution of our vector and its safety profile.

We anticipate receiving feedback from the FDA on the design of our potentially pivotal Phase 2/3 clinical trial in the second half of 2022, and we intend to initiate the clinical trial in the first half of 2023.

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 Friedreich's ataxia, Alzheimer's disease, CLN2 Batten 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 Novartis, PTC Therapeutics, Inc., Aavanti Bio, Inc., Lacerta Therapeutics, Inc. and those being developed in collaborations between Voyager Therapeutics, Inc. and Neurocrine Biosciences, Inc. and between Takeda Pharmaceutical Company Limited and StrideBio, Inc. Among other treatment modalities for Friedreich's ataxia, we are aware that Larimar Therapeutics, Inc. is developing a clinical stage product candidate, CTI-1601, and that Reata Pharmaceuticals, Inc. is developing late-clinical-stage candidate omaveloxolone.

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 toxic variant while expressing the 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 aducanumab (Aduhelm), which was recently approved under accelerated approval and will require confirmatory data to verify clinical benefit. Finally, we are aware that Voyager Therapeutics, Inc. and Taysha Gene Therapies, Inc. are pursuing Alzheimer's disease treatments and have early-stage discovery efforts ongoing based on vectorized antibodies and tau-specific miRNA shuttles, respectively.

With respect to LX1004, the FDA approved BioMarin Pharmaceutical, Inc.'s treatment for CLN2 Batten disease, cerliponase alfa (Brineura) in 2017. Brineura is an enzyme replacement therapy which requires chronic twice monthly infusions directly into the CSF via intraventricular injections. In addition to Brineura, REGENXBIO Inc. is developing two preclinical gene therapy candidates to treat the CNS and ocular manifestations of CLN2 Batten disease.

With respect to LX2020, Tenaya Therapeutics Inc. is developing a preclinical AAV-based gene therapy candidate designed to deliver a functional *PKP2* gene to patients with ARVC.

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.

Manufacturing

To date, we have utilized an adherent approach to manufacture vector for non-clinical and clinical use. However, in October 2021, we engaged with a CDMO for process development and cGMP manufacture of our gene therapy candidates using a suspension baculovirus/Sf9 expression system. We have partnered 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 our viral vectors. Our new manufacturing platform infects 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, allowing for efficient AAV purification. We expect that cGMP manufacture of our product candidates using this platform will begin in the second half of this year.

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 will own the intellectual property created by our manufacturing process development activities or have the ability to license it, and we will maintain the option to transfer our manufacturing process to other CDMOs in the future and/or to our own potential manufacturing facility to ensure ongoing redundancy and reliability of production.

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, for example 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 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 products 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 February 1, 2022, we in-license two U.S. patents, five pending U.S. non-provisional patent applications, two pending U.S. provisional applications, three pending PCT applications, two foreign patents and 16 pending foreign applications.

We in-license from Cornell University and Adverum Biotechnologies, Inc. two U.S. patents, two pending U.S. non-provisional patent applications, one pending U.S. provisional application, two foreign patents and 10 pending foreign applications that relate to our LX2006 cardiac FA program. The patents we in-license outside of the United States are granted in Mexico and New Zealand. The patent applications we in-license outside of the United States are pending in Australia, Brazil, China, Eurasia, Europe, Israel, India, Mexico and South Africa.

Not accounting for any patent term adjustment, regulatory extension or terminal disclaimers, and assuming that all annuity and/or maintenance fees are paid timely, these patents, and if granted these patent applications, 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 in-license from Cornell University one pending PCT application that relates to our LX1020 Alzheimer's APOE2+/E4- program. Not accounting for any patent term adjustment, regulatory extension or terminal disclaimers, and assuming that all annuity and/or maintenance fees are paid timely, if granted, patent applications claiming the priority to this PCT application, will expire in 2040. This pending PCT application discloses and/or contains 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 discloses and/or contains claims to methods of producing and methods of treatment using the APOE2+/APOE4- vector.

We in-license from Cornell University one pending PCT application that relates to our LX1021 Alzheimer's Christchurch program. Not accounting for any patent term adjustment, regulatory extension or terminal disclaimers, and assuming that all annuity and/or maintenance fees are paid timely, if granted, patent applications claiming the priority to this PCT application, will expire in 2040. This pending PCT application discloses and/or contains composition-of-matter claims to a vector encoding a mutated human apolipoprotein E protein, and discloses and/or contains claims to methods of producing and methods of treatment using the mutated human apolipoprotein E vector.

License 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, is based on the transferred IND from Cornell University, uses any licensed materials, or is produced using a licensed method, and to practice certain licensed technology, in all cases, for all human and non-human prophylactic and therapeutic uses. 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 First License Agreement, Cornell University assigned to us an IND for the use of AAVrh10.hAPOE vector to treat *APOE4* homozygous patients who are at risk or have Alzheimer's disease. Cornell University reserves the rights to practice the patents and technology for non-commercial educational and non-commercial research purposes and to publish and disseminate information about inventions included in the licensed technology and licensed patents.

We are obligated to diligently proceed with the development, manufacture, and sale of licensed products, to raise certain amounts within specified timeframes, to achieve certain development milestones within specified timeframes, to meet agreed minimum-spend requirements, and to meet other diligence obligations.

Under the Cornell First License Agreement, we paid Cornell University a license issue and assignment fee in the high six digits. 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 Product. We are also obligated to pay milestone payments up to the high seven digits for each portfolio upon the

achievement of specified development and regulatory milestones by a product in such portfolio. 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 a small amount. 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 of the last valid claim in the licensed patent, (b) the expiration of regulatory exclusivity, and (c) the launch of a generic equivalent in such country. We are also obligated to pay Cornell University a percentage of sublicensing fees ranging from low double digits to mid-double digits, decreasing over time based on when we grant a sublicense.

We may terminate the Cornell First License Agreement or any portfolio thereunder at any time upon 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, is based on the transferred IND from Cornell University, uses any licensed materials, or is produced using a licensed method, and to practice certain licensed technology, in all cases, for all human and non-human prophylactic and therapeutic uses. The technology under the license includes portfolios for CLN2 Batten Disease and 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. Additionally, under the Second License Agreement, Cornell University assigned to us an IND for the use of AAVrh.10CUhCLN2 to treat children with infantile neuronal ceroid lipofuscinosis (also called CLN2 Batten disease). Cornell University reserves the rights to practice the patents and technology for non-commercial educational and non-commercial research purposes and to publish and disseminate information about inventions included in the licensed technology and licensed patents.

We are obligated to diligently proceed with the development, manufacture, and sale of licensed products, to raise certain amounts within specified timeframes, to achieve certain development milestones within specified timeframes, to meet agreed minimum-spend requirements, and to meet other diligence obligations.

Under the Cornell Second License Agreement, we paid Cornell University a license issue and assignment fee in the high six digits. 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. We are also obligated to pay milestone payments up to mid to low seven digits in two portfolios and in the mid to high six digits for a third portfolio upon the achievement of specified development and regulatory milestones by a product in such portfolio. We are obligated to pay Cornell University a flat royalty in the mid-single-digits based 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 a small amount. 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 of the last valid claim in the licensed patent, (b) the expiration of regulatory exclusivity, and (c) the launch of a generic equivalent in such country. We are also obligated to pay Cornell University a percentage of sublicensing fees ranging from low double digits to mid-double digits, decreasing over time based on when we grant a sublicense.

We may terminate the Cornell Second License Agreement or any portfolio thereunder at any time upon 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 of any remaining licensed product.

License agreement with Adverum

In January 2021, we entered into a license agreement with Adverum Biotechnologies, Inc., or the Adverum Agreement, pursuant to which 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 Product.

Under the 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 and up to $49 million in commercialization and sales milestones for the Products. We are obligated to pay Adverum tiered royalties ranging from a rate in the high single-digits to 10% 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 regulatory exclusivity, and (c) ten years after the first commercial sale of such Product in such country.

The 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 Adverum Agreement at any time upon advance written notice to Adverum. In addition, subject to certain conditions, either we or Adverum may terminate the 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 Adverum Agreement if we challenge the validity of any licensed patents. Upon expiration of the Adverum Agreement, the license becomes royalty-free, irrevocable and perpetual. Upon termination of the Adverum Agreement, all licenses and rights granted by either party will terminate.

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 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 GLP regulations;
- submission to the FDA of an IND, which must become effective before human clinical trials may begin;
- approval by an 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, GCPs 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 after completion of all pivotal clinical trials;
- 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 GCPs;
- 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 FDA as part of an IND to the FDA. An IND is a request for authorization from the FDA to administer an investigational new drug 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 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 must become effective before human clinical trials may begin. The IND 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 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 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 GCPs, 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 must be made for each successive clinical trial 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.

In addition to the submission of an IND to the FDA before initiation of a clinical trial in the United States, certain human clinical trials involving recombinant or synthetic nucleic acid molecules are subject to oversight of IBCs as set forth in the National Institutes for Health, or NIH, Guidelines for Research Involving Recombinant or Synthetic Nucleic Acid Molecules, or NIH Guidelines. Under the NIH Guidelines, recombinant and synthetic nucleic acids are defined as: (i) molecules that are constructed by joining nucleic acid molecules and that can replicate in a living cell (i.e., recombinant nucleic acids); (ii) nucleic acid molecules that are chemically or by other means synthesized or amplified, including those that are chemically or otherwise modified but can base pair with naturally occurring nucleic acid molecules (i.e., synthetic nucleic acids); or (iii) molecules that result from the replication of those described in (i) or (ii). Specifically, under the NIH Guidelines, supervision of human gene transfer trials includes evaluation and assessment by an IBC, a local institutional committee that reviews and oversees research utilizing recombinant or synthetic nucleic acid molecules at that institution. The IBC assesses the safety of the research and identifies any potential risk to public health or the environment, and such review may result in some delay before initiation of a clinical trial. While the NIH Guidelines are not mandatory unless the research in question is being conducted at or sponsored by institutions receiving NIH funding of recombinant or synthetic nucleic acid molecule research, many companies and other institutions not otherwise subject to the NIH Guidelines voluntarily follow them. 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. If a foreign clinical trial is not conducted under an IND, 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 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 GCP 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, 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 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 after the FDA accepts the application for filing. In both standard and priority reviews, the review process is often 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 GCPs. 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 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 review programs for qualifying product candidates. The fast track program is intended to expedite or facilitate the process for reviewing 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 a RMAT concurrently with or at any time after 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 and accelerated approval. 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.

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, that is reasonably likely to predict an effect on irreversible morbidity or mortality 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 to verify and describe the anticipated effect on irreversible morbidity or mortality or other clinical benefit. 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 promotional materials intended for dissemination or publication within 120 days of marketing approval, which could adversely impact the timing of the commercial launch of the product.

Fast track designation, breakthrough therapy designation, RMAT designation, priority review and accelerated approval 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 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 than 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 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.

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 of 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 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 tosupport dosing and administration for each pediatric subpopulation for which the product is safe and effective. The FDA may grant deferrals for 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 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 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 ACA includes a subtitle called the Biologics Price Competition and Innovation Act (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 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 is subject to significant uncertainty.

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. Such laws include, without limitation: the U.S. federal Anti-Kickback Statute, the civil False Claims Act, 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. The U.S. federal Anti-Kickback Statute has been interpreted to apply to arrangements between pharmaceutical manufacturers on one hand and prescribers, purchasers and others on the other hand. 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. Failure to meet all of the requirements of a particular applicable statutory exception or regulatory safe harbor does not make the conduct per se illegal

under the U.S. federal Anti-Kickback Statute. Instead, the legality of the arrangement will be evaluated on a case-by-case basis based on a cumulative review of all of its facts and circumstances. 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. 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. A claim includes "any request or demand" for money or property presented to the U.S. government. For example, pharmaceutical and other healthcare companies have been prosecuted under these laws for allegedly providing free product to customers with the expectation that the customers would bill federal programs for the product.

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. 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 CMS information related to payments or other transfers of value made to physicians (defined to include doctors, dentists, optometrists, podiatrists and chiropractors) and teaching hospitals, as well as ownership and investment interests held by physicians and their immediate family members. Effective January 1, 2022, these reporting obligations were extended to payments and other transfers of value made during the previous year to certain non-physician providers, including physician assistants, nurse practitioners, clinical nurse specialists, anesthesiologist assistants, certified registered nurse anesthetists and certified nurse midwives.

We are also 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. 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. However, on January 11, 2022, CMS proposed to cover FDA-approved monoclonal antibodies directed against amyloid for the treatment of Alzheimer's disease only for patients enrolled in some CMS-approved randomized controlled trials and trials supported by the National Institutes of Health. Since CMS's proposal, there has been pushback from drug manufacturers and patient advocacy groups to expand Medicare coverage of this class of drug for patients beyond those enrolled in these trials. Thus, it is unclear whether Medicare coverage and reimbursement will be available for any of our product candidates for treatment of Alzheimer's disease, once approved.

Furthermore, any 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.

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 March 2010, substantially changed the way healthcare is financed by both governmental and private insurers in the United States, and significantly affected the pharmaceutical industry.

The ACA contains a number of provisions of particular import to the pharmaceutical and biotechnology industries, including, but not limited to, those governing enrollment in federal healthcare programs, a new methodology by which rebates owed by manufacturers under the Medicaid Drug Rebate Program are calculated for drugs that are inhaled, infused, instilled, implanted or injected, and annual fees based on pharmaceutical companies' share of sales to federal healthcare programs. Since its enactment, there have been judicial, Congressional and executive branch challenges to certain aspects of the ACA. For example, Congress has considered legislation that would repeal or repeal and replace all or part of the ACA. While Congress has not passed comprehensive repeal legislation, it has enacted laws that modify certain provisions of the ACA such as removing penalties, which started on January 1, 2019, for not complying with ACA's individual mandate to carry health insurance, delaying the implementation of certain ACA-mandated fees, and increasing the point-of-sale discount that is owed by pharmaceutical manufacturers who participate in Medicare Part D. On June 17, 2021 the U.S. Supreme Court dismissed a challenge on procedural grounds that argued the ACA is unconstitutional in its entirety because the "individual mandate" was repealed by Congress. Thus, the ACA will remain in effect in its current form. Further, prior to the U.S. Supreme Court ruling, on January 28, 2021, President Biden issued an executive order that initiated a special enrollment period for purposes of obtaining health insurance coverage through the ACA marketplace, which began on February 15, 2021 and remained open until August 15, 2021. The executive order also instructed certain governmental agencies to review and reconsider their existing policies and rules that limit access to healthcare, including among others, reexamining Medicaid demonstration projects and waiver programs that include work requirements, and policies that create unnecessary barriers to obtaining access to health insurance coverage through Medicaid or the ACA. It is possible that the ACA will be subject to judicial or Congressional challenges in the future. Other legislative changes have been proposed and adopted since the ACA was enacted, including aggregate reductions of Medicare payments to providers of 2% per fiscal year and reduced payments to several types of Medicare providers. These reductions went into effect in April 2013 and, due to subsequent legislative amendments to the statute, will remain in effect through 2031 unless additional action is taken by Congress. However, pursuant to the CARES Act, these Medicare sequester reductions will be suspended from May 1, 2020 through December 31, 2021 due to the COVID-19 pandemic. In addition, Congress is considering additional health reform measures as part of the budget reconciliation process. It is also possible that additional governmental action is taken in response to the COVID-19 pandemic.

Moreover, 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. For example, in July 2021, the Biden administration released an executive order, "Promoting Competition in the American Economy," with multiple provisions aimed at prescription drugs. In response to President Biden's executive order, on September 9, 2021, HHS released a Comprehensive Plan for Addressing High Drug Prices that outlines principles for drug pricing reform and sets out a variety of potential legislative policies that Congress could pursue as well as potential administrative actions HHS can take to advance these principles. No legislation or administrative actions have been finalized to implement these principles. 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.

Facilities

Our principal executive office is located in New York, New York, where we lease a workspace for a small number of team members under a lease that can be terminated with 30 days' notice of the annual renewal period.

In January 2022, we entered into a lease for new facilities with a total of approximately 15,839 square feet of office and laboratory space that we will use for our administrative, research and development and other activities beginning in May 2021, under a lease that currently expires in early 2029. We believe that our new facilities are sufficient to meet our current needs and that suitable additional space will be available as and when needed.

Employees and human capital resources

As of December 31, 2021, we had 24 full-time employees and no part-time employees. Of our 24 full- and part-time employees, 18 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.

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, 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. We are not currently subject to any material legal proceedings.

Management

Executive officers and directors

The following table provides information regarding our current executive officers and directors, including their ages as of December 31, 2021:

Name	Age	Position(s)
Executive Officers		
R. Nolan Townsend	42	Chief Executive Officer and Director
Jay A. Barth, M.D.	58	Executive Vice President and Chief Medical Officer
Paul McCormac, Ph.D.	51	Chief Technical Officer
Non-Employee Directors		
Steven Altschuler, M.D.	68	Chairman of the Board of Directors
Mette Kirstine Agger	57	Director
Paula HJ Cholmondeley	74	Director
Bernard Davitian	61	Director
Reinaldo Diaz	67	Director

(1) Member of the audit committee.
(2) Member of the compensation committee.
(3) Member of the nominating and corporate 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 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.

Jay A. Barth, M.D. has served as our Executive Vice President and Chief Medical Officer since October 2020. Prior to joining Lexeo, Dr. Barth served as the Chief Medical Officer of Amicus Therapeutics, Inc., a global pharmaceutical company, from March 2014 to October 2020. In that role, Dr. Barth led an organization focused on rare disease and gene therapy and oversaw Phase 1 through Phase 4 development and clinical and regulatory activities leading to FDA, EMA, and global regulatory approvals. Dr. Barth began his biopharmaceutical career in 1998 at Eisai Medical Research, Inc., and since then held positions of increasing responsibility in clinical development at several companies. Immediately prior to Amicus Therapeutics, Inc., Dr. Barth was Senior Vice President, Clinical Development, at PTC Therapeutics, Inc. Dr. Barth received a B.A. in History from Columbia University and an M.D. from the University of Pennsylvania School of Medicine.

Paul McCormac, Ph.D. has served as our Chief Technical Officer since December 2021, prior to this he served as our Senior Vice President of Technical Operations since March 2021. Prior to joining Lexeo, Dr. McCormac was an Executive Director, Medicinal Sciences Category Lead for Pfizer Rare Disease, a unit of Pfizer, Inc., a global pharmaceutical company, from October 2016 until March 2021. In that role, he managed chemistry, manufacturing, and controls, or CMC, for product development with a focus on Pfizer's gene therapy CMC strategy. Prior to this, he served as Director/Team Leader for Pfizer's Bio-Manufacturing Sciences group from November 2008 until October 2016. Dr. McCormac received a B.S. in Analytical Science and a Ph.D. in Organic Chemistry from Dublin City University and completed a post-doctoral fellowship at Queen's University Belfast.

Non-employee 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 since January 2020 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 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. Since 2009, Ms. Agger has served as a Managing Partner of Lundbeckfonden Ventures, a life sciences venture capital fund and, with its affiliates, a holder of more than 5% of our voting securities. Prior to joining Lundbeckfonden Ventures, Ms. Agger co-founded 7TM A/S, a biotech company engaged in therapeutic drug discovery and development, in 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 scPharmaceuticals Inc., a public pharmaceutical company, since March 2014 and Imara Inc., a public biopharmaceutical company, since January 2016. 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, since 2002. Ms. Cholmondeley holds an M.S. and a B.S. in Accounting from the Wharton School of the University of Pennsylvania. We believe 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.

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, 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. 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 has served as a Venture Partner at Longitude Capital, a healthcare venture capital firm since 2015, and he currently serves as the Chief Business Officer of Opna-Immuno Oncology, SA, 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 Auven 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., where he chairs the compensation committee. Mr. Diaz received a B.A. in Economics 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.

Board composition

Our business and affairs are managed under the direction of our board of directors, which currently consists of five members. Our directors were elected to, and currently serve on, the board pursuant to a voting agreement among us and all of our stockholders and voting rights granted by our current amended and restated certificate of incorporation. The voting agreement will terminate upon the closing of this offering, after which there will be no further contractual obligations regarding the election of our directors.

In accordance with our amended and restated certificate of incorporation that will be in effect upon the closing of this offering, our board of directors will be divided into three classes, each of which will consist, as nearly as possible, of one-third of the total number of directors constituting our entire board and which will serve staggered three-year terms. At each annual meeting of stockholders, the successors to directors whose terms then expire will be elected to serve from the time of election and qualification until the third annual meeting following election. Our directors will be divided among the three classes as follows:

- Class I, which will consist of and , and their terms will expire at our first annual meeting of stockholders to be held after the closing of this offering;
- Class II, which will consist of and , and their terms will expire at our second annual meeting of stockholders to be held after the closing of this offering; and

- Class III, which will consist of and , and their terms will expire at our third annual meeting of stockholders to be held after the closing of this offering.

Our amended and restated bylaws, which will become effective upon the closing of this offering, will provide that the authorized number of directors may be changed only by resolution approved by a majority of our board of directors. Any additional directorships resulting from an increase in the number of directors will be distributed among the three classes so that, as nearly as possible, each class will consist of one-third of the directors. The division of our board of directors into three classes with staggered three-year terms may delay or prevent a change of our management or a change of control.

Director independence

Applicable Nasdaq rules, or the Nasdaq Listing Rules, require a majority of a listed company's board of directors to be comprised of independent directors within one year of listing. In addition, the Nasdaq Listing Rules require that, subject to specified exceptions, each member of a listed company's audit, compensation and nominating and corporate governance committees be independent and that audit committee members also satisfy independence criteria set forth in Rule 10A-3 under the Exchange Act of 1934, as amended, or the Exchange Act. The Nasdaq independence definition includes a series of objective tests, such as that the director is not, and has not been for at least three years, one of our employees, that neither the director nor any of his family members has engaged in various types of business dealings with us and that the director is not associated with the holders of more than 5% of our common stock. In addition, under applicable Nasdaq rules, a director will only qualify as an "independent director" if, in the opinion of the listed company's board of directors, that person does not have a relationship that would interfere with the exercise of independent judgment in carrying out the responsibilities of a director.

Our board of directors has determined that all of our directors other than , representing of our directors, are "independent directors" as defined under applicable Nasdaq rules. In making such determination, our board of directors considered the current and prior relationships that each such director has with our company and all other facts and circumstances that our board of directors deemed relevant in determining his or her independence, including the beneficial ownership of our capital stock by each director and the transactions described in the section titled "Certain Relationships and Related Party Transactions."

There are no family relationships among any of our directors or executive officers.

Role of the board in risk oversight

One of the key functions of our board of directors is informed oversight of our risk management process. Our board of directors does not have a standing risk management committee, but rather administers this oversight function directly through the board of directors as a whole, as well as through various standing committees of our board of directors that address risks inherent in their respective areas of oversight. In particular, our board of directors is responsible for monitoring and assessing strategic risk exposure. Following the completion of this offering, we intend for our audit committee to have the responsibility to consider and discuss our major financial risk exposures and the steps our management has taken to monitor and control these exposures, including guidelines and policies to govern the process by which risk assessment and management is undertaken. The audit committee will also monitor compliance with legal and regulatory requirements.

Board committees

Our board of directors has established an audit committee, compensation committee and a nominating and corporate governance committee, each of which operate pursuant to a committee charter. Our board of directors

may establish other committees to facilitate the management of our business. The composition and functions of each committee are described below.

Audit committee

Upon the completion of this offering, our audit committee will consist of , and , with serving as chair of the audit committee. Our board of directors has determined that each of these individuals meets the independence requirements of Rule 10A-3 under the Securities Exchange Act of 1934, or the Exchange Act, and the applicable listing standards of Nasdaq. Each member of our audit committee can read and understand fundamental financial statements in accordance with Nasdaq audit committee requirements. Our board of directors has also determined that qualifies as an audit committee financial expert within the meaning of SEC regulations and meets the financial sophistication requirements of the Nasdaq Listing Rules. In arriving at these determinations, the board has examined each audit committee member's scope of experience and the nature of their prior and/or current employment.

The functions of this committee include, among other things:

- helping our board of directors oversee our corporate accounting and financial reporting processes;
- managing 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;
- discussing the scope and results of the audit with the independent registered public accounting firm, and reviewing, with management and the independent accountants, our interim and year-end operating results;
- developing procedures for employees to submit concerns anonymously about questionable accounting or audit matters;
- reviewing related person transactions;
- obtaining and reviewing 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; and
- approving or, as permitted, pre-approving, audit and permissible non-audit services to be performed by the independent registered public accounting firm.

We believe that the composition and functioning of our audit committee will comply with all applicable SEC and Nasdaq rules and regulations. We intend to comply with future requirements to the extent they become applicable to us.

Compensation committee

Upon the completion of this offering, our compensation committee will consist of , and , with serving as chair of the compensation committee. Each of these individuals is a non-employee director, as defined in Rule 16b-3 promulgated under the Exchange Act and is an "outside director," as defined pursuant to Section 162(m) of the Internal Revenue Code of 1986, as amended, or the Code. Our board of directors has determined that each of these individuals is ''independent" as defined under the applicable listing standards of Nasdaq, including the standards specific to members of a compensation committee. The functions of this committee include, among other things:

- reviewing, modifying and approving (or if it deems appropriate, making recommendations to the full board of directors regarding) our overall compensation strategy and policies;

- making recommendations to the full board of directors regarding the compensation and other terms of employment of our executive officers;
- reviewing and making recommendations to the full board of directors regarding performance goals and objectives relevant to the compensation of our executive officers and assessing their performance against these goals and objectives;
- reviewing and approving (or if it deems it appropriate, making recommendations to the full board of directors regarding) the equity incentive plans, compensation plans and similar programs advisable for us, as well as modifying, amending or terminating existing plans and programs;
- evaluating risks associated with our compensation policies and practices and assessing whether risks arising from our compensation policies and practices for our employees are reasonably likely to have a material adverse effect on us;
- reviewing and making recommendations to the full board of directors regarding the type and amount of compensation to be paid or awarded to our non-employee board members;
- establishing policies with respect to votes by our stockholders to approve executive compensation to the extent required by Section 14A of the Exchange Act and, if applicable, determining our recommendations regarding the frequency of advisory votes on executive compensation;
- reviewing and assessing the independence of compensation consultants, legal counsel and other advisors as required by Section 10C of the Exchange Act;
- administering our equity incentive plans;
- establishing policies with respect to equity compensation arrangements;
- reviewing the competitiveness of our executive compensation programs and evaluating the effectiveness of our compensation policy and strategy in achieving expected benefits to us;
- reviewing and making recommendations to the full board of directors regarding the terms of any employment agreements, severance arrangements, change in control protections and any other compensatory arrangements for our executive officers;
- reviewing with management and approving our disclosures under the caption “Compensation Discussion and Analysis” in our periodic reports or proxy statements to be filed with the SEC, to the extent such caption is included in any such report or proxy statement;
- preparing the report that the SEC requires in our annual proxy statement; and
- reviewing and evaluating on an annual basis the performance of the compensation committee and the compensation committee charter.

We believe that the composition and functioning of our compensation committee will comply with all applicable SEC and Nasdaq rules and regulations. We intend to comply with future requirements to the extent they become applicable to us.

Nominating and corporate governance committee

Upon the completion of this offering, our nominating and corporate governance committee will consist of , and , with serving as chair of the nominating and corporate governance committee. Our board of directors has determined that each of these individuals is ‘‘independent”

as defined under the applicable listing standards of Nasdaq and SEC rules and regulations. The functions of this committee include, among other things:

- identifying, reviewing and evaluating candidates to serve on our board of directors;
- determining the minimum qualifications for service on our board of directors;
- evaluating director performance on the board and applicable committees of the board and determining whether continued service on our board is appropriate;
- evaluating, nominating and recommending individuals for membership on our board of directors;
- evaluating nominations by stockholders of candidates for election to our board of directors;
- considering and assessing the independence of members of our board of directors;
- developing a set of corporate governance policies and principles and recommending to our board of directors any changes to such policies and principles;
- reviewing and making recommendations to the board of directors with respect to management succession planning;
- considering questions of possible conflicts of interest of directors as such questions arise; and
- reviewing and evaluating on an annual basis the performance of the nominating and corporate governance committee and the nominating and corporate governance committee charter.

We believe that the composition and functioning of our nominating and corporate governance committee will comply with all applicable SEC and Nasdaq rules and regulations. We intend to comply with future requirements to the extent they become applicable to us.

Compensation committee interlocks and insider participation

None of our directors who serve as a member of our compensation committee is, or has at any time during the past year been, one of our officers or employees. None of our executive officers currently serves, or in the past year has served, as a member of the board of directors or compensation committee of any other entity that has one or more executive officers serving on our board of directors or compensation committee.

Code of business conduct and ethics

Effective upon the closing of this offering, we will adopt a Code of Business Conduct and Ethics, or the Code of Conduct, applicable to all of our employees, executive officers and directors. This includes our principal executive officer, principal financial officer and principal accounting officer or controller, or persons performing similar functions. Following the closing of this offering, the full text of the Code of Conduct will be available on our website at *www.lexeotx.com*. We intend to post on our website all disclosures that are required by law or the listing standards of the Nasdaq Stock Market concerning any amendments to, or waivers from, any provision of the Code of Conduct. Information contained on, or that can be accessed through, our website is not incorporated by reference into this prospectus, and you should not consider information on our website to be part of this prospectus.

Non-employee director compensation

The following table shows information concerning the compensation that our non-employee directors earned during the last completed fiscal year ended December 31, 2021. A director who is also our employee receives no additional compensation for his or her services as a director.

The following table shows information concerning the compensation that our non-employee directors earned during the last completed fiscal year ended December 31, 2021. A director who is also our employee receives no additional compensation for his or her services as a director.

Name	Fees Earned or Paid in Cash ($)(1)	Option Awards ($)(2)(3)	Total ($)
Sandip Agarwala(4)	—	—	—
Mette Kirstine Agger	—	—	—
Steven Altschuler, M.D.	150,000	154,583	304,583
Paula HJ Cholmondeley	5,344	33,113	38,547
Bernard Davitian	—	—	—
Reinaldo Diaz(5)	—	—	—

(1) Represents actual fees paid in fiscal year 2021. For Dr. Altschuler, this includes fees paid for board services . For Ms. Cholmondeley, this includes fees paid for board services. Mr. Agarwala, Ms. Agger, Mr. Davitian and Mr. Diaz did not receive cash compensation for their service to us as non-employee directors in fiscal year 2021.

(2) Option awards in this column are reported at the aggregate grant date fair value in accordance with FASB ASC Topic 718. Option awards under this column are discussed in greater detail below.

(3) As of December 31, 2021, our then non-employee directors held the following option awards:

		Option Awards			
Name	Grant Date	Unexercisable	Exercisable	Exercise Price ($)	Expiration Date
Sandip Agarwala	—	—	—	—	—
Mette Kirstine Agger	—	—	—	—	—
Steven Altschuler, M.D.	2/16/2021	—	1,189,097	0.22	2/15/2031
Paula HJ Cholmondeley	11/15/2021	—	165,565	0.46	11/14/2031
Bernard Davitian	—	—	—	—	—
Reinaldo Diaz	—	—	—	—	—

(4) Sandip Agarwala resigned from our board of directors effective January 28, 2022.

(5) Reinaldo Diaz was appointed to our board of directors effective February 2, 2022.

Stock option awards

Certain non-employee directors serving on our board of directors received stock option awards during 2021. On February 16, 2021, Steven Altschuler, M.D. received a grant of 1,189,097 stock options, with a vesting start date of January 5, 2021, and on November 15, 2021, Paula Cholmondeley received a grant of 165,565 stock options. The stock options granted to Dr. Altschuler and Ms. Cholmondeley were exercisable on the date of grant and vest with respect to 25% of the underlying shares on the first anniversary of the vesting start date and thereafter in equal monthly installments over three years.

Executive compensation

Our named executive officers for the period ended December 31, 2021, consisting of our principal executive officer and the next two most highly compensated executive officers, were:

- R. Nolan Townsend, who currently serves as our Chief Executive Officer and as a member of our board of directors;
- Jay A. Barth, M.D., who currently serves as our Executive Vice President and Chief Medical Officer; and
- Paul McCormac, Ph.D., who currently serves as our Chief Technical Officer.

Summary compensation table

The following table sets forth information regarding compensation awarded to, earned by and paid to our named executive officers with respect to the period ended December 31, 2021.

Name and Principal Position	Year	Salary ($)	Bonus ($)	Stock Awards ($)(1)	Option Awards ($)(1)	Non-Equity Incentive Plan Compensation ($)(2)	All Other Compensation ($)	Total ($)
R. Nolan Townsend	2021	410,833	—	—	1,382,160	267,375	1,500	2,061,869
Chief Executive Officer and Director								
Jay A. Barth, M.D.	2021	399,167	—	—	816,045	151,200	—	1,366,411
Executive Vice President and Chief Medical Officer								
Paul McCormac, Ph.D.	2021	256,944	—	—	330,724	86,625	1,125	675,419
Chief Technical Officer								

(1) Stock and option awards in these columns are reported at the aggregate grant date fair value in accordance with FASB ASC Topic 718. Assumptions used in the calculation of these amounts are included in Note to our audited financial statements included elsewhere in this prospectus.

(2) Non-equity incentive plan compensation amounts represent actual amounts earned during 2021, but paid in 2022. The amount paid to each named executive officer is equal to a percentage of the executive's target annual bonus pursuant to the applicable employment agreement.

Outstanding equity awards at December 31, 2021

The following table sets forth certain information about outstanding equity awards granted to our named executive officers that were outstanding as of December 31, 2021.

			Option Awards(1)			
Name	Grant Date	Vesting Commencement Date	Number of Securities Underlying Unexercised Options Exercisable	Number of Securities Underlying Unexercised Options Unexercisable	Option Exercise Price ($)	Option Expiration Date
R. Nolan Townsend	2/16/2021	11/21/2020	1,627,848(2)	—	0.22	2/15/2031
Chief Executive Officer	11/15/2021	08/11/2021	5,500,000(3)	—	0.46	11/14/2031
Jay A. Barth, M.D.	2/16/2021	11/21/2020	1,783,645(4)	—	0.22	2/15/2031
Executive Vice President and Chief Medical Officer	11/15/2021	08/11/2021	2,920,855(3)	—	0.46	11/14/2021
Paul McCormac, Ph.D.	3/22/2021	3/22/2021	487,350(3)	—	0.22	3/21/2031
Chief Technical Officer	11/15/2021	08/11/2021	1,336,844(3)	—	0.46	11/14/2021

(1) All of the option awards were granted under the Existing Plan. All of the option awards were granted with a per share exercise price equal to the fair market value of one share of our common stock on the date of grant.

(2) All of the 2,170,463 shares originally subject to this option were exercisable as of the date of grant, and 542,615 shares were exercised on May 14, 2021. Of the total shares subject to this option, 25% will vest one year after the vesting commencement date, and the remaining shares vest in 36 equal monthly installments thereafter, subject to continuous service through each applicable vesting date. All unvested shares will vest immediately prior to the consummation of a change in control of the Company. As of December 31, 2021, 587,833 shares subject to this option have vested, of which 542,615 shares have been exercised and 45,218 vested shares remain unexercised.

(3) All of the shares subject to these options were exercisable as of the date of grant. 25% of the total shares subject to these options will vest one year after the vesting commencement date, and the remaining shares vest in 36 equal monthly installments thereafter, subject to continuous service through each applicable vesting date. All unvested shares will vest immediately prior to the consummation of a change in control of the Company. As of December 31, 2021, zero shares subject to these options have vested.

(4) All of the shares subject to this option were exercisable as of the date of grant. Of the total shares subject to this option, 25% will vest one year after the vesting commencement date, and the remaining shares vest in 36 equal monthly installments thereafter, subject to continuous service through each applicable vesting date. All unvested shares will vest immediately prior to the consummation of a change in control of the Company. As of December 31, 2021, 483,070 shares subject to this option have vested.

Agreements with our named executive officers

We have entered into employment agreements with each of our named executive officers, setting forth the terms and conditions of such executive's employment with us, as described below. The employment agreements generally set forth the executive officer's initial base salary, annual bonus opportunity, eligibility for employee benefits and termination benefits, and restrictive covenants. The key terms of the employment agreements are described below. In connection with this offering, we intend to enter into amended and restated employment agreements with each of our named executive officers.

R. Nolan Townsend

We entered into an employment agreement with Mr. Townsend, our Chief Executive Officer, in January 2020 that governs the current terms of his employment with us. In 2021, Mr. Townsend received a base salary of $410,833. Pursuant to the employment agreement, Mr. Townsend is eligible to receive a target bonus equal to 50% of his annual base salary based upon Mr. Townsend's achievement of certain performance objectives agreed to by us in writing by or before January 31 of each calendar year during his term of employment. For 2021, Mr. Townsend received a bonus of $267,375. Mr. Townsend was granted a number of incentive units of LEXEO Therapeutics, LLC equal to 5% of the total number in the aggregate of LEXEO Therapeutics, LLC's common units and incentive units outstanding on January 1, 2020, subject to the terms and conditions of applicable award agreements. Mr. Townsend is also eligible for benefits upon an involuntary termination of his employment with us, as described in more detail below under the section titled "—Potential payments upon termination or change in control." In addition, Mr. Townsend is eligible to participate in our regular health insurance and other employee benefit plans, as described below as described in more detail below under the section titled "—Other compensation and benefits."

Jay A. Barth, M.D.

We entered into an employment agreement with Dr. Barth, our Executive Vice President and Chief Medical Officer, in October 2020 that governs the current terms of his employment with us. In 2021, Dr. Barth received a base salary of $399,167. Pursuant to the employment agreement, Dr. Barth is eligible to receive a target bonus equal to 40% of his annual base salary with the exact amount to be determined by our board of directors, and for 2021 Dr. Barth received a bonus of $151,200. Pursuant to the employment agreement, Dr. Barth was also granted incentive equity awards in connection with the company's closing of certain funding rounds. The initial incentive award vests, subject to Dr. Barth's continuous employment with us and the terms and conditions of applicable award agreements, with respect to 25% of the grant on the one-year anniversary of the effective date of the employment agreement, and with respect to the remaining 75% of the grant, monthly thereafter in substantially equal installments for 36 months. Dr. Barth is also eligible for benefits upon an involuntary termination of his employment with us, as described in more detail below under the section titled "—Potential

payments upon termination or change in control." In addition, Dr. Barth is eligible to participate in our regular health insurance and other employee benefit plans, as described below as described in more detail below under the section titled "—Other compensation and benefits."

Paul McCormac, Ph.D.

We entered into an employment agreement with Dr. McCormac, our Chief Technical Officer, in February 2021 that governs the current terms of his employment with us. In 2021, Dr. McCormac received a base salary of $256,944. Pursuant to the employment agreement, Dr. McCormac is eligible to receive a target bonus equal to 30% of his annual base salary with the exact amount to be determined by our board of directors, and, for 2021, Dr. McCormac received a bonus of $86,625. Dr. McCormac was granted 487,350 options in connection with his employment with us. The options vest, subject to Dr. McCormac's continuous employment with us and the terms and conditions of applicable award agreements, with respect to 25% of the grant on the one-year anniversary of the date of grant, and with respect to the remaining 75% of the grant, monthly thereafter in substantially equal installments for 36 months. Dr. McCormac is also eligible for benefits upon an involuntary termination of his employment with us, as described in more detail below under the section titled "—Potential payments upon termination or change in control." In addition, Dr. McCormac is eligible to participate in our regular health insurance and other employee benefit plans, as described below as described in more detail below under the section titled "—Other compensation and benefits."

Potential payments upon termination or change in control

Pursuant to Mr. Townsend's employment agreement, upon a termination of his employment by us without "cause" (and not due to death or disability) or by Mr. Townsend for "good reason" (as those terms are defined in the employment agreements) and subject to his executive of a valid release of claims in favor of the company, Mr. Townsend is entitled to the following payments: (i) any earned but unpaid annual bonus from the year prior to the year in which his termination occurs, (ii) accrued vacation, (iii) reimbursement for COBRA premiums, if applicable, for Mr. Townsend and his dependents for up to 15 months, (iv) continuation of base salary for a period of 15 months, (v) continued vesting of equity awards as provided in the applicable award agreements, (vi) any accrued base salary or annual bonus unpaid as of the termination date, (vii) any vested benefits earned under any employee benefits plan, and (viii) any unreimbursed business expenses incurred prior to the termination date, if otherwise reimbursable.

For the purpose of Mr. Townsend's employment agreement, "cause" generally means (1) the willful or repeated failure or refusal by the executive to substantially perform his material duties under the employment agreement (other than such failure resulting from the executive's incapacity due to physical or mental illness; and it being agreed that failure of the company to achieve operating results or similar poor performance of the company shall not, in and of themselves, be deemed a failure to perform the executive's duties); (2) the plea of guilty or no contest or conviction or indictment of the executive of any felony or any crime involving moral turpitude (other than misdemeanors for DUI or DWI); (3) the executive's commission of an act of fraud, embezzlement or material misappropriation of assets involving us or any of our subsidiaries or affiliates; (4) any material breach by the executive of his obligations under the employment agreement; (5) any breach of any fiduciary duty owed to us or any of ours subsidiaries or affiliates under the law or under the employment agreement or any other written agreement or written covenant with us unless the action giving rise to such breach was taken at the direction of our founder or our board of directors, as applicable, or was based on the executive's good faith belief that such action was in the best interests of the company and/or our subsidiaries; or (6) any willful action under the law or under the employment agreement or any other written agreement or written covenant with the company that is reasonably likely, in the good faith opinion of a majority of our board of directors (other than the

executive), to result in material harm to the reputation or business prospects of the company or any of our subsidiaries or affiliates.

For the purpose of Mr. Townsend's employment agreement, "good reason" generally means, without the executive's written consent (1) a reduction in base salary, target annual performance or benefits opportunity amount (in each case other than a reduction applied consistently to all senior executives of the company), (2) a material diminution of the executive's job responsibilities, authority or title (regardless of whether any of the company's governing documents provide the company and/or our board of directors with the ability to materially reduce such job responsibilities, authority or title); (3) the executive is required to report to any entity or person other than, prior to the date on which the majority of our board of directors consists of independent directors, our founder and on and after the date on which a majority of our board of directors consists of independent directors, our board of directors or a committee thereof, (4) prior to an initial public offering of the company's or our successor's or parent's equity securities, the executive is removed by the members of the company from our board of directors other than for "cause" or "disability" (as those terms are defined in the employment agreement), (5) relocation of the executive's principal worksite of more than 35 miles from New York, New York, excluding business travel or (6) the company's material breach of the employment agreement or any other agreement between us and the executive, including any agreement awarding the executive equity in the company.

Pursuant to Dr. Barth's employment agreement, upon a termination of his employment by us without "cause" (and not due to death or "disability") or by Dr. Barth for "good reason" (as those terms are defined in the employment agreements) and subject to his execution of a valid release of claims in favor of the company, Dr. Barth is entitled to the following payments: (i) any earned but unpaid annual bonus from the year prior to the year in which his termination occurs, (ii) a pro rata target annual bonus for the year of termination, (iii) reimbursement for COBRA premiums, if applicable, for Dr. Barth and his dependents for up to 12 months, and (iv) continuation of base salary for a period of 12 months. In addition, all of Dr. Barth's incentive equity awards granted prior to the Company's initial public offering pursuant to his employment agreement vest in connection with a Change in Control as defined in the Existing Plan.

Pursuant to Dr. McCormac's employment agreement, upon a termination of his employment by us without "cause" (and not due to death or "disability") or by Dr. McCormac for "good reason" (as those terms are defined in the employment agreements) and subject to his execution of a valid release of claims in favor of the company, Dr. McCormac is entitled to the following payments: (i) any earned but unpaid annual bonus from the year prior to the year in which his termination occurs, (ii) accrued vacation, (iii) reimbursement for COBRA premiums, if applicable, for Dr. McCormac and his dependents for up to 6 months, and (iv) continuation of base salary for a period of 11 months.

For the purposes of Dr. Barth's and Dr. McCormac's employment agreements, "cause" generally means (1) the willful failure or refusal by the executive to substantially perform his duties under the employment agreement (other than such failure resulting from the executive's incapacity due to physical or mental illness); (2) the plea of guilty or no contest or conviction or indictment of the executive of any felony or any crime involving moral turpitude; (3) the executive's commission of an act of fraud, embezzlement or material misappropriation of assets involving the company or any of our subsidiaries or affiliates; (4) any material breach by the executive of his obligations under the employment agreement; (5) any breach of any fiduciary duty owed to the company or any of our subsidiaries or affiliates under the law or under the employment agreement or any other written agreement or written covenant with us or any of our subsidiaries or affiliates; or (6) any willful action that is reasonably likely, in the reasonable good faith opinion of our board or directors, to result in material harm to the reputation of business prospects of the company or any of our subsidiaries or affiliates.

For the purpose of Dr. Barth's employment agreement, "good reason" generally means, without the executive's written consent, (1) a material reduction in his base salary (other than a reduction applied consistently to all senior executives of the company), (2) a material diminution in the executive's reporting responsibilities, duties, authorities or responsibilities, (3) a relocation of the executive's principal location of employment of more than 35 miles, provided that such relation materially increases the executive's daily commute, or (4) the company's other material breach of this agreement.

For the purpose of Dr. McCormac's employment agreement, "good reason" generally means, without the executive's written consent, (1) a material reduction in his base salary (other than a reduction applied consistently to all senior executives of the company), or (2) the company's material breach of the employment agreement.

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 our employees, including our named executive officers, 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. For the year ended December 31, 2021, we provided matching contributions under our 401(k) plan representing 5.9% of total contributions to the 401(k) plan.

Equity incentive plans

2022 equity incentive plan

Our board of directors adopted the 2022 Equity Incentive Plan, or 2022 Plan, in 2022, and our stockholders approved the 2022 Plan in 2022. The 2022 Plan will become effective upon the execution of the underwriting agreement for this offering. Once the 2022 Plan becomes effective, no further grants will be made under the Existing Plan.

Types of Awards. Our 2022 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 2022 Plan is shares, which is the sum of (i) new shares, plus (ii) the Existing Plan's available reserve, plus (iii) the number of returning shares, 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 2022 Plan will automatically increase on January 1 of each year, beginning on January 1, 2023, and continuing through and including January 1, 2032, by % 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 2022 Plan is shares.

Shares issued under our 2022 Plan will be authorized but unissued or reacquired shares of common stock. Shares subject to awards granted under our 2022 Plan that expire or terminate without being exercised in full, or that are paid out in cash rather than in shares, will not reduce the number of shares available for issuance under our 2022 Plan. Additionally, shares issued pursuant to awards under our 2022 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 2022 Plan.

The maximum number of shares of common stock subject to stock awards granted under the 2022 Plan or otherwise during any calendar year beginning in 2022 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 $ 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, $.

Plan administration. Our board of directors, or a duly authorized committee of our board, may administer our 2022 Plan. We expect that our board of directors will delegate concurrent authority to administer our 2022 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 2022 Plan.

In addition, subject to the terms of the 2022 Plan, the administrator also has the power to modify outstanding awards under our 2022 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 2022 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 2022 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 2022 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-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 2022 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 2022 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 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 2022 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 2022 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 2022 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 2022 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.

In the event of a corporate transaction, any stock awards outstanding under the 2022 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 2022 Plan other than by will, the laws of descent and distribution, or as otherwise provided under our 2022 Plan.

Plan amendment or termination. Our board has the authority to amend, suspend, or terminate our 2022 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 adopted our 2022 Plan. No awards may be granted under our 2022 Plan while it is suspended or after it is terminated.

2022 employee stock purchase plan

Our board of directors adopted the the ESPP on , 2022, and our stockholders adopted the ESPP on , 2022. The ESPP will become effective upon the execution of the underwriting agreement for this offering. The purpose of the 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 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 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 ESPP is shares. The number of shares of common stock reserved for issuance under our ESPP will automatically increase on January 1 of each calendar year, beginning on January 1, 2023 and continuing through and including January 1, 2032, by the lesser of (1) % of the total number of shares of common stock outstanding on December 31 of the preceding calendar year, (2) shares and (3) a number of shares determined by our board. Shares subject to purchase rights granted under our ESPP that terminate without having been exercised in full will not reduce the number of shares available for issuance under our ESPP.

Plan administration. Our board, or a duly authorized committee thereof, will administer our ESPP. We anticipate that our board will delegate concurrent authority to administer our ESPP to the compensation committee under the terms of the compensation committee's charter. The 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 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 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 ESPP and may contribute, normally through payroll deductions, with a maximum dollar amount as designated by the board. Unless otherwise determined by the administrator, common stock will be purchased for the accounts of employees participating in the 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 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 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 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 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 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 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 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.

ESPP amendment or termination. The administrator has the authority to amend or terminate our 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 ESPP as required by applicable law or listing requirements.

2021 equity incentive plan

Our board of directors adopted the Existing Plan in February 2021, and our stockholders approved the Existing Plan in September 2021.

Stock awards. The Existing 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 Existing Plan. As of December 31, 2021, options to purchase shares of our common stock were outstanding with a weighted-average exercise price of $ per share and restricted stock awards to purchase shares of our common were outstanding, and shares of our common stock remained available for future awards under the Existing Plan.

Share reserve. Subject to certain capitalization adjustments, the aggregate number of shares of our common stock that has been reserved for issuance pursuant to stock awards under the Existing Plan is shares.

Shares subject to stock awards granted under our Existing 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 Existing 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 Existing 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 Existing Plan.

Plan administration. Our board of directors administers and interprets the provisions of the Existing 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 our Existing 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 Existing Plan and awards granted thereunder, and prescribe, amend, modify, and rescind or terminate rules and regulations for the administration of the Existing Plan. Under the Existing Plan, the administrator may, with the consent of the majority of holders of shares entitled to vote, implement a plan to amend outstanding awards to provide for a lower exercise price or issue new awards in exchange for the surrender and cancellation of outstanding awards.

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 Existing 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 Existing 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 Existing 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 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 Existing 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 Existing Plan, the administrator will adjust the number, kind and class of securities that may be delivered under the Existing 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 Existing 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.

Under the Existing Plan, an 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 may at any time terminate, amend or suspend the Existing Plan and all outstanding options or restricted stock awards upon a dissolution or liquidation of us.

Limitations on liability and indemnification matters

Upon the closing of this offering, our amended and restated certificate of incorporation will contain provisions that limit the liability of our current and former directors for monetary damages to the fullest extent permitted by Delaware law. Delaware law provides that directors of a corporation will not be personally liable for monetary damages for any breach of fiduciary duties as directors, except liability for:

- any breach of the director's duty of loyalty to the corporation or its stockholders;
- any act or omission not in good faith or that involves intentional misconduct or a knowing violation of law;
- unlawful payments of dividends or unlawful stock repurchases or redemptions as provided in Section 174 of the Delaware General Corporation Law; or
- any transaction from which the director derived an improper personal benefit.

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 that will be in effect upon the closing of this offering will provide that we are required to indemnify our directors to the fullest extent permitted by Delaware law. Our amended and restated bylaws will 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 will also provide our board of directors with discretion to indemnify our officers and employees when determined appropriate by the board.

We have entered into indemnification agreements with each of our directors, and we expect to enter into amended and restated indemnification agreements with each of our directors and enter into indemnification agreements with each of our executive officers prior to the closing of this offering. 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.

Rule 10b5-1 sales plans

Our directors and executive officers may adopt written plans, known as Rule 10b5-1 plans, in which they will contract with a broker to buy or sell shares of our common stock on a periodic basis. Under a Rule 10b5-1 plan, a broker executes trades pursuant to parameters established by the director or officer when entering into the plan, without further direction from them. The director or officer may amend a Rule 10b5-1 plan in some circumstances and may terminate a plan at any time. Our directors and executive officers also may buy or sell additional shares outside of a Rule 10b5-1 plan when they are not in possession of material nonpublic information subject to compliance with the terms of our insider trading policy. Prior to 180 days after the date of this offering, subject to early termination, the sale of any shares under such plans would be prohibited by the lock-up agreement that the director or officer has entered into with the underwriters.

Certain relationships and related party transactions

The following is a description of transactions since January 1, 2019 to which we have been a participant in which the amount involved exceeded or will exceed $120,000, and in which any of our directors, executive officers or holders of more than 5% of our voting securities, or any members of their immediate family, had or will have a direct or indirect material interest, other than compensation arrangements which are described under “Executive Compensation.”

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 will provide 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, or the Services. 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 1,791,822 shares of our common stock at an exercise price per share of $0.22 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% will vest on the one-year anniversary of the closing of our third tranche of financing from the sale of our Series A preferred convertible stock, or August 20, 2022, and the remaining shares subject to the option will vest in 36 substantially equal installments thereafter. 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.

Private placements of our securities

Series A convertible preferred stock financing

In November 2020, we entered into a preferred stock purchase agreement with certain investors, including beneficial owners of greater than 5% of our capital stock and affiliates of members of our board of directors. In connection with the initial closing of our Series A convertible preferred stock, we sold an aggregate of 34,495,725 shares of our Series A convertible preferred stock at a purchase price of $1.00 (including approximately $4 million in principal and accrued interest on outstanding convertible promissory notes issued to certain investors, the conversion of which resulted in issuance of 4,495,729 shares of our Series A convertible preferred stock) for aggregate gross proceeds of approximately $34 million. In July 2021, we sold an aggregate of 50,999,997 shares of our Series A convertible preferred stock at a purchase price of $1.00 for aggregate gross proceeds of approximately $51 million.

The table below sets forth the aggregate number of shares of Series A convertible preferred stock issued to our related parties in this financing:

Name	Series A Convertible Preferred Stock (#)	Aggregate Purchase Price ($)
Entities affiliated with Janus Henderson Investors(1)	10,000,000	10,000,000
Longitude Venture Partners IV, L.P.(2)	18,999,999	18,999,999
Lundbeckfond Invest A/S(3)	13,999,999	13,999,999
Omega Fund VI, L.P.(4)	14,999,999	14,999,999
PBM LEX Holdings, LLC(5)	11,999,999	11,999,999

(1) Entities affiliated with Janus Henderson Investors hold more than 5% of our voting securities. Consists of (i) 3,643,715 shares of Series A preferred stock purchased by Janus Henderson Global Life Sciences Fund, (ii) 3,466,664 shares of Series A preferred stock purchased by Janus Henderson Biotech Innovation Master Fund Limited, (iii) 2,660,459 shares of Series A preferred stock purchased by Janus Henderson Capital Funds PLC on behalf of its Series Janus Henderson Global Life Sciences Fund and (iv) 229,162 shares of Series A preferred stock purchased by Janus Henderson Horizon Fund—Biotechnology Fund.

(2) Longitude Venture Partners IV, L.P., or LVP IV, holds more than 5% of our capital stock prior to this offering. Longitude Capital Partners IV, LLC is the general partner of LVP IV. Reinaldo Diaz was designated to serve as a member of our board of directors by LVP IV.

(3) Lundbeckfond Invest A/S, which is wholly owned by the Lundbeck Foundation, holds more than 5% of our capital stock prior to this offering. Mette Kirstine Agger is the Managing Partner of Lundbeckfonden Ventures, 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.

(4) Omega Fund VI, L.P., or Omega Fund, holds more than 5% of our capital stock prior to this offering. Omega Fund VI GP, L.P., or Omega GP LP, is the sole general partner of the Omega Fund, and Omega Fund VI GP Manager, Ltd. is the sole general partner of Omega GP LP. Bernard Davitian was designated to serve as a member of our board of directors by Omega Fund.

(5) PBM LEX Holdings, LLC, or PBM LEX, holds more than 5% of our capital stock prior to this offering.

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 capital stock, 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.

The table below sets forth the aggregate number of shares of Series B convertible preferred stock issued to our related parties in this financing:

Name	Series B Convertible Preferred Stock (#)	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 capital stock prior to this offering. 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 capital stock prior to this offering. Longitude Capital Partners IV, LLC is the general partner of LVP IV. 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 capital stock prior to this offering. Mette Kirstine Agger is the Managing Partner of Lundbeckfonden Ventures, 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) Mutual Fund Series Trust, on behalf of Eventide Healthcare & Life Sciences Fund, holds more than 5% of our capital stock prior to this offering.

(6) Omega Fund holds more than 5% of our capital stock prior to this offering. Omega GP LP is the sole general partner of the Omega Fund, and Omega Fund VI GP Manager, Ltd. is the sole general partner of Omega GP LP. Bernard Davitian was designated to serve as a member of our board of directors by Omega Fund.

(7) All shares are held by Paul McCormac, Ph.D., our Chief Technical Officer.

Investors' rights, voting and right of first refusal agreements

In connection with the sales of convertible preferred stock described above, we entered into an amended and restated investors' rights agreement, an amended and restated voting agreement and an amended and restated right of first refusal and co-sale agreement containing registration rights, information rights, voting rights and rights of first refusal, among other things, with the holders of our convertible preferred stock, including funds affiliated with D1 Capital Partners, Eventide Healthcare & Life Sciences Fund, Janus Henderson Investors, Longitude Capital, Lundbeckfond Invest A/S, Omega Funds and PBM Capital. These agreements will terminate upon the closing of this offering, except for the registration rights granted under our amended and restated investors' rights agreement, as more fully described in the section of this prospectus titled "Description of Capital Stock—Registration Rights."

Employment arrangements

We have entered into employment agreements or offer letter agreements with certain of our executive officers. For more information regarding such employment agreements, see "Executive Compensation—Employment Agreements with our Named Executive Officers."

Indemnification agreements

Our amended and restated certificate of incorporation that will be in effect upon the closing of this offering will contain provisions limiting the liability of directors, and our amended and restated bylaws will provide that we will indemnify each of our directors to the fullest extent permitted under Delaware law. Our amended and restated certificate of incorporation and amended and restated bylaws will also provide our board of directors with discretion to indemnify our officers and employees when determined appropriate by the board.

In addition, we have entered into indemnification agreements with each of our directors, and we expect to enter into amended and restated indemnification agreements with each of our directors and enter into indemnification agreements with each of our executive officers prior to the closing of this offering. For more information regarding these agreements, see "Executive Compensation—Limitations on Liability and Indemnification Matters."

Related person transaction policy

Prior to this offering, we have not had a formal policy regarding approval of transactions with related parties. In connection with this offering, we have adopted a related person transaction policy that sets forth our procedures for the identification, review, consideration and approval or ratification of related person transactions, which policy will become effective immediately upon the execution of the underwriting agreement for this offering. For purposes of our policy only, a related person transaction will be 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 in which the amount involved exceeds $120,000. Transactions involving compensation for services provided to us as an employee or director will not be covered by this policy. A related person will be 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.

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 will 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 that we expect to adopt prior to the closing of this offering, our employees and directors will 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 related person transactions, our audit committee, or other independent body of our board of directors, will take into account the relevant available facts and circumstances including:

- the risks, costs and benefits to us;
- 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;
- the availability of other sources for comparable services or products; and
- the terms available to or from, as the case may be, unrelated third parties or to or from employees generally.

The policy will require that, in determining whether to approve, ratify or reject a related person transaction, our audit committee, or other independent body of our board of directors, must consider, in light of known circumstances, whether the transaction is in, or is not inconsistent with, our best interests and those of our stockholders, as our audit committee, or other independent body of our board of directors, determines in the good faith exercise of its discretion.

All of the transactions described in this section were entered into prior to the adoption of this policy. Although we have not had a written policy for the review and approval of transactions with related persons, our board of directors has historically reviewed and approved any transaction where a director or officer had a financial interest, including the transactions described above. 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.

Principal stockholders

The following table sets forth information regarding beneficial ownership of our capital stock by:

- each person, or group of affiliated persons, known by us to beneficially own more than 5% of our common stock;
- each of our directors;
- our named executive officers; and
- all of our current executive officers and directors as a group.

We have determined beneficial ownership in accordance with the rules of the SEC. Under these rules, beneficial ownership includes any shares of common stock as to which the individual or entity has sole or shared voting power or investment power. Applicable percentage ownership is based on shares of common stock outstanding as of , 2022, after giving effect to the conversion of all of our preferred stock. In computing the number of shares beneficially owned by an individual or entity and the percentage ownership of that person, shares of common stock subject to options held by such person that are currently exercisable or will become exercisable within 60 days of , 2022 are considered outstanding, although these shares are not considered outstanding for purposes of computing the percentage ownership of any other person.

Unless noted otherwise, the address of all listed stockholders is c/o Lexeo Therapeutics, Inc., 420 East 29th Street, Floor 14, New York, New York 10016.

Except as indicated by the footnotes below, we believe, based on information furnished to us, that each of the stockholders listed has sole voting and investment power with respect to the shares beneficially owned by the stockholder unless noted otherwise, subject to community property laws where applicable.

	Number of Shares Beneficially Owned	Percentage of Shares Beneficially Owned	
Name of Beneficial Owner		**Before Offering**	**After Offering**
Greater than 5% stockholders			
D1 Master Holdco I LLC(1)			%
PBM LEX Holdings, LLC(2)			
Entities affiliated with Janus Henderson Investors(3)			
Longitude Venture Partners IV, L.P.(4)			
Lundbeckfond Invest A/S(5)			
Mutual Fund Series Trust, on Behalf of Eventide Healthcare & Life Sciences Fund(6)			
Omega Fund VI, L.P(7)			
Ronald G. Crystal, M.D. and affiliates(8)			
Named Executive Officers and Directors			
R. Nolan Townsend(9)			
Jay A. Barth, M.D.(10)			
Paul McCormac, Ph.D.(11)			
Mette Kirstine Agger			
Steven Altschuler, M.D.(12)			
Paula HJ Cholmondeley(13)			
Bernard Davitian			
Reinaldo Diaz			
All current executive officers and directors as a group (eight persons)(14)			

* Represents beneficial ownership of less than one percent.

(1) All of the shares are held by D1 Master Holdco I LLC. Consists of shares of common stock issuable upon conversion of Series B convertible preferred stock. D1 Capital Partners L.P., or D1 Management Company, is a registered investment adviser and serves as the investment manager of private investment vehicles and accounts, including D1 Master Holdco I LLC, and may be deemed to beneficially own the shares of common stock held by D1 Master Holdco I LLC. Daniel Sundheim indirectly controls the D1 Management Company and may be deemed to beneficially own the shares of common stock held by D1 Master Holdco I LLC. The business address of each of D1 Master Holdco I LLC, D1 Capital Partners L.P. and Daniel Sundheim is 9 West 57th Street, 36th Floor, New York, New York 10019.

(2) Consists of shares of common stock issuable upon conversion of Series A convertible preferred stock held by PBM LEX Holdings, LLC. PBM LEX Holdings, LLC is majority owned by PBM Capital Investments II, LLC, which is managed by PBM Capital Group, LLC. Paul B. Manning is the Chief Executive Officer of PBM Capital Group, LLC, and has sole voting and investment power with respect to the shares held by PBM LEX Holdings, LLC. The business address for each person and entity named in this footnote is 200 Garrett Street, Suite S, Charlottesville, Virginia 22902.

(3) Consists of (i) shares of common stock issuable upon conversion of Series A convertible preferred stock held by Janus Henderson Biotech Innovation Master Fund Limited, (ii) shares of common stock issuable upon conversion of Series A convertible preferred stock held by Janus Henderson Horizon Fund – Biotechnology Fund, (iii) shares of common stock issuable upon conversion of Series A convertible preferred stock held by Janus Henderson Capital Funds PLC On Behalf Of Its Series Janus Henderson Global Life Sciences Fund, (iv) shares of common stock issuable upon conversion of Series B convertible preferred stock held by Janus Henderson Capital Funds PLC On Behalf Of Its Series Janus Henderson Global Life Sciences Fund, (v) shares of common stock issuable upon conversion of Series A convertible preferred stock held by Janus Henderson Global Life Sciences Fund and (vi) shares of common stock issuable upon conversion of Series B convertible preferred stock held by Janus Henderson Global Life Sciences Fund. Such shares owned by the funds may be deemed to be beneficially owned by, Janus Henderson Investors US LLC, or Janus, an investment adviser registered under the Investment Advisers Act of 1940, who acts as investment adviser for the funds set forth above and has the ability to make decisions with respect to the voting and disposition of the shares subject to the oversight of the board of trustees (or similar entity) of each fund. Under the terms of its management contract with each fund, Janus has overall responsibility for directing the investments of the fund in accordance with the fund's investment objective, policies and limitations. Each fund has one or more portfolio managers appointed by and serving at the pleasure of Janus who makes decisions with respect to the disposition of the Shares. The address for Janus and each of the foregoing entities is 151 Detroit Street, Denver, Colorado 80206.

(4) All of the shares are held by Longitude Venture Partners IV, L.P. (LVP IV). Consists of (i) shares of common stock issuable upon conversion of Series A convertible preferred stock and (ii) shares of common stock issuable upon conversion of Series B convertible preferred stock. 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 for this entity is 2740 Sand Hill Road, 2nd Floor, Menlo Park, California 94025.

(5) All of the shares are held by Lundbeckfond Invest A/S. Consists of (i) shares of common stock issuable upon conversion of Series A convertible preferred stock and (ii) shares of common stock issuable upon conversion of Series B convertible preferred stock. Lundbeckfond Invest A/S is wholly-owned by the Lundbeck Foundation, and the board of directors of the Lundbeckfond Foundation consists of Steffen Kragh, Lars Holmqvist, Susanne Krüger Kjær, Michael Kjær, Peter Schütze, Gunhild Waldemar, Svend Andersen, Ludovic Tranholm Otterbein, Vagn Flink Møller Pedersen and Kristian Funding Andersen. The board of directors of the Lundbeck Foundation also serve as the board members of Lundbeckfond Invest A/S. No individual member of the Lundbeckfond Invest A/S board of directors is deemed to hold any beneficial ownership or reportable pecuniary interest in the shares held by Lundbeckfond Invest A/S. The board of directors of Lundbeckfond Invest A/S makes decisions with respect to investments made by Lundbeckfond Invest A/S, and the board of directors of Lundbeckfond Invest A/S and Lene Skole, the chief executive officer of Lundbeckfond Invest A/S, may be deemed to share voting and investment authority over the shares held by Lundbeckfond Invest A/S. The address of Lundbeckfond Invest A/S is Scherfigsvej 7 DK-2100, Copenhagen Ø, Denmark.

(6) All of the shares are held by Mutual Fund Series Trust, On Behalf of Eventide Healthcare & Life Sciences Fund. Consists of shares of common stock issuable upon conversion of Series B convertible preferred stock. 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 for this entity is c/o Eventide Asset Management, LLC, One International Place, Suite 4210, Boston, Massachusetts 02110.

(7) All of the shares are held by Omega Fund. Consists of (i) shares of common stock issuable upon conversion of Series A convertible preferred stock and (ii) shares of common stock issuable upon conversion of Series B convertible preferred stock. Omega Fund GP Manager, Ltd., or Omega Ltd., is the sole general partner of Omega Fund VI GP, L.P., or Omega GP, which is the sole general partner of Omega Fund; and each of Omega Ltd. and Omega GP may be deemed to own beneficially the shares held by Omega Fund. Claudio Nessi, Otello Stampacchia and Anne-Mari Paster are the managing 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, and Ms. Paster, and 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 this entity is 888 Boylston Street, Suite 1111, Boston, Massachusetts 02199.

(8) Consists of (i) shares held by Ronald G. Crystal, M.D., (ii) shares held by the Ronald G. Crystal 2021 Grantor Retained Annuity Trust over which Dr. Crystal retains sole voting and dispositive power and (iii) shares subject to options held by Dr. Crystal that are exercisable within 60 days of , 2022.

(9) Consists of (i) shares of common stock and (ii) shares subject to options that are exercisable within 60 days of , 2022.

(10) Includes shares subject to options that are exercisable within 60 days of , 2022.

(11) Includes shares subject to options that are exercisable within 60 days of , 2022.

(12) Includes shares subject to options that are exercisable within 60 days of , 2022.

(13) Includes shares subject to options that are exercisable within 60 days of , 2022.

(14) Consists of (i) shares of common stock and (ii) shares subject to options that are exercisable within 60 days of , 2022.

Description of capital stock

The following description of our capital stock, certain provisions of our amended and restated certificate of incorporation and amended and restated bylaws, as each will be in effect following the completion of this offering, and certain provisions of Delaware law are summaries. You should also refer to the amended and restated certificate of incorporation and the amended and restated bylaws, which are filed as exhibits to the registration statement of which this prospectus is part.

General

Upon the completion of this offering, our amended and restated certificate of incorporation will authorize us to issue up to shares of common stock, $0.0001 par value per share, and shares of preferred stock, $0.0001 par value per share, all of which shares of preferred stock will be undesignated. Our board of directors may establish the rights and preferences of the preferred stock from time to time. As of December 31, 2021, there were shares of Series A convertible preferred stock outstanding and shares of Series B convertible preferred stock outstanding, held by stockholders of record. All currently outstanding shares of convertible preferred stock will be converted into an aggregate of shares of common stock upon the closing of this offering.

Common stock

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.

Preferred stock

Following the closing of this offering, our board of directors will have the authority under our amended and restated certificate of incorporation, without further action by our stockholders, to issue up to shares of preferred stock in one or more series, to establish from time to time the number of shares to be included in each such series, to fix the rights, preferences and privileges of the shares of each wholly unissued series and any qualifications, limitations or restrictions thereon, and to increase or decrease the number of shares of any such series, but not below the number of shares of such series then outstanding.

Our board of directors may authorize the issuance of preferred stock with voting or conversion rights that could adversely affect the voting power or other rights of the holders of our common stock. The purpose of authorizing our board of directors to issue preferred stock and determine its rights and preferences is to eliminate delays associated with a stockholder vote on specific issuances. The issuance of preferred stock, while providing flexibility in connection with possible acquisitions and other corporate purposes, could, among other things, have the effect of delaying, deferring or preventing a change in control of us and may adversely affect the market price of our common stock and the voting and other rights of the holders of our common stock. It is not possible to state the actual effect of the issuance of any shares of preferred stock on the rights of holders of common stock until the board of directors determines the specific rights attached to that preferred stock.

We have no present plans to issue any shares of preferred stock following completion of this offering.

Options and restricted stock awards

As of December 31, 2021, there were options to purchase shares of common stock outstanding and no shares of common stock had been issued pursuant to restricted stock awards. For additional information regarding the terms of the Existing Plan, see “Executive Compensation—Equity Incentive Plans.”

Registration rights

We, the holders of our existing convertible preferred stock and certain holders of our existing common stock have entered into an amended and restated investors’ rights agreement. The registration rights provisions of this agreement provide those holders with demand, piggyback and Form S-3 registration rights with respect to the shares of common stock currently held by them and issuable to them upon conversion of our convertible preferred stock in connection with our initial public offering. These shares are collectively referred to herein as registrable securities.

Demand registration rights

At any time beginning 180 days following the effective date of the registration statement of which this prospectus is a part, the holders of a majority of registrable securities then outstanding have the right to demand that we file a Form S-1 registration statement covering registrable securities then outstanding having an aggregate offering price in excess of $10 million. These registration rights are subject to specified conditions and limitations, including the right of the underwriters, if any, to limit the number of shares included in any such registration under specified circumstances. Upon such a request, we are required to effect a Form S-1 registration statement as soon as practicable, but in any event no later than 60 days after the date such request is given by the initiating holders. An aggregate of shares of common stock will be entitled to these demand registration rights.

Piggyback registration rights

If we propose to register any of our securities under the Securities Act either for our own account or for the account of other stockholders, the holders of registrable securities will each be entitled to notice of the

registration and will be entitled to include their shares of common stock in the registration statement. These piggyback registration rights are subject to specified conditions and limitations, including the right of the underwriters to limit the number of shares included in any such registration under specified circumstances. An aggregate of shares of common stock will be entitled to these piggyback registration rights.

Registration on Form S-3

At any time after we become eligible to file a registration statement on Form S-3, the holders of at least 10% of registrable securities then outstanding will be entitled to request to have such shares registered by us on a Form S-3 registration statement. These Form S-3 registration rights are subject to other specified conditions and limitations, including the condition that the anticipated aggregate offering price, net of certain selling expenses, is at least $1 million. Upon receipt of this request, the holders of registrable securities will each be entitled to participate in this registration. An aggregate of shares of common stock will be entitled to these Form S-3 registration rights.

Expenses of registration

We are required to pay all expenses, including reasonable fees and expenses of one counsel to represent the selling stockholders, relating to any demand, piggyback or Form S-3 registration, other than underwriting discounts and commissions, stock transfer taxes and any additional fees of counsel for the selling stockholders, subject to specified conditions and limitations. We are not required to pay registration expenses if a demand registration request is withdrawn at the request of a majority of holders of registrable securities to be registered, unless holders of a majority of the registrable securities agree to forfeit their right to one demand registration.

The amended and restated investors' rights agreement contains customary cross-indemnification provisions, pursuant to which we are obligated to indemnify the selling stockholders in the event of material misstatements or omissions in the applicable registration statement attributable to us, and the selling stockholders are obligated to indemnify us for material misstatements or omissions in the registration statement attributable to them, subject to certain limitations.

Termination of registration rights

The registration rights granted under the investors' rights agreement will terminate with respect to any particular stockholder upon the earlier of (a) the closing of a deemed liquidation event, as defined in our certificate of incorporation, (b) with respect to each stockholder, at such time such stockholder is able to sell all of its shares pursuant to Rule 144 or another similar exemption under the Securities Act during a three-month period without registration and (c) the fifth anniversary of the closing of this offering or such later date that is 180 days following the expiration of all deferrals of our obligations with respect to any registration rights exercised by holders of registrable securities as of such fifth anniversary.

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 to be in effect upon the completion of this offering, or our restated certificate, will provide 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 restated certificate and our amended and restated bylaws to be effective upon the completion of this offering, or our restated bylaws, will also provide 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 restated certificate of incorporation and amended and restated bylaws our stockholders will 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 restated certificate and restated bylaws will 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 restated bylaws will 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 restated bylaws will 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 restated certificate and restated bylaws will provide that the stockholders cannot amend many of the provisions described above except by a vote of 66-2/3% or more of our outstanding common stock.

As described in "—Preferred Stock" above, our restated certificate will give our board of directors the authority, without further action by our stockholders, to issue up to shares of preferred stock in one or more series, with any rights, preferences and privileges as they may designate, including the right to approve an acquisition or other change in control.

The combination of these provisions will make 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 will provide 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 will further provide 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 . The transfer agent’s address is .

Listing

We intend to apply for listing of our common stock on the Nasdaq Global Market under the trading symbol “LXEO.”

Shares eligible for future sale

Prior to this offering, 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 prevailing from time to time. As described below, only a limited number of shares will be available for sale shortly after this offering 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.

Based on the number of shares outstanding as of December 31, 2021, upon the closing of this offering and assuming no exercise of the underwriters' option to purchase additional shares, shares of common stock will be outstanding, assuming no outstanding options are exercised. All of the shares of common stock sold in this offering will be freely tradable without restrictions or further registration under the Securities Act, except for any shares sold to our "affiliates," as that term is defined under Rule 144 under the Securities Act. The remaining shares of common stock held by existing stockholders are "restricted securities," as that term is defined in Rule 144 under the Securities Act. Restricted securities may be sold in the public market only if registered or if their resale qualifies for exemption from registration described below under Rule 144 promulgated under the Securities Act or another available exemption.

As a result of the lock-up agreements described below and the provisions of Rules 144 and 701 under the Securities Act, the shares of common stock that will be deemed restricted securities after this offering will be available for sale in the public market as follows:

- none of the existing shares will be eligible for immediate sale upon the completion of this offering; and
- shares will be eligible for sale in the public market upon expiration of lock-up agreements 180 days after the date of this prospectus, subject in certain circumstances to the volume, manner of sale and other limitations under Rule 144 and Rule 701 under the Securities Act, which are summarized below.

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 shares immediately after the completion of this offering based on the number of shares outstanding as of December 31, 2021; 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 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 in the section titled “Underwriting” and will become eligible for sale upon the expiration of the restrictions set forth in those agreements.

Form S-8 registration statements

We intend to file one or more registration statements on Form S-8 under the Securities Act to register all shares of common stock subject to outstanding stock options and common stock issued or issuable under our equity plans. We expect to file the registration statement covering shares offered pursuant to our stock plans as soon as practicable after the closing of this offering, permitting the resale of such shares by non-affiliates in the public market without restriction under the Securities Act and the sale by affiliates in the public market, subject to compliance with the resale provisions of Rule 144 and expiration or release from the terms of the lock-up agreements described above.

Lock-up agreements

We, our executive officers and directors and substantially all of the holders of our common stock outstanding on the date of this prospectus have entered into lock-up agreements with the underwriters or otherwise agreed, subject to limited exceptions, that we and they will not, not to 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 SVB Securities LLC for a period of 180 days from the date of this prospectus.

In addition to the restrictions contained in the lock-up agreements described above, we have 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 the date of this prospectus.

Registration rights

Upon the closing of this offering, the holders of shares of our common stock, including common stock issuable upon the conversion of our preferred stock, or their transferees, will be 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.

Material U.S. federal income tax consequences to non-U.S. holders

The following is a summary of certain material U.S. federal income tax consequences to non-U.S. holders (as defined below) of the ownership and disposition of our common stock offered pursuant to this prospectus. This discussion is not a complete analysis of all potential U.S. federal income tax consequences relating thereto, does not address the potential application of the Medicare contribution tax on net investment income, the alternative minimum tax, or the special tax accounting rules under Section 451(b) of the Code, and does not address any U.S. federal non-income tax consequences such as estate or gift tax consequences or any tax consequences arising under any state, local, or non-U.S. tax laws. This discussion is based on the Code and applicable Treasury Regulations promulgated thereunder, judicial decisions and published rulings, and administrative pronouncements of the IRS all as in effect as of the date hereof. These authorities are subject to differing interpretations and may change, possibly retroactively, resulting in U.S. federal income tax consequences different from those discussed below. We have not requested, and do not intend to request, a ruling from the IRS with respect to the U.S. federal income tax consequences discussed below, and there can be no assurance that the IRS or a court will agree with such tax consequences.

This discussion is further limited to non-U.S. holders that hold our common stock as a "capital asset" within the meaning of Section 1221 of the Code (generally, property held for investment). This discussion also does not address all U.S. federal income tax consequences that may be relevant to a particular non-U.S. holder in light of such non-U.S. holder's particular circumstances. Finally, this discussion does not address any specific facts or circumstances that may be relevant to non-U.S. holders subject to special rules under U.S. federal income tax laws, including:

- certain former citizens or long-term residents of the United States;
- partnerships, S corporations, or other entities or arrangements treated as partnerships, pass-through entities, or disregarded entities (including hybrid entities) for U.S. federal income tax purposes (and investors therein);
- "controlled foreign corporations" within the meaning of Section 957(a) of the Code;
- "passive foreign investment companies" within the meaning of Section 1297(a) of the Code;
- corporations that accumulate earnings to avoid U.S. federal income tax;
- banks or other financial institutions;
- real estate investment trusts or regulated investment companies;
- insurance companies;
- brokers or dealers in securities;
- persons that have elected to mark securities to market;
- tax-exempt organizations (including private foundations), governmental organizations, or international organizations;
- tax-qualified retirement plans;
- persons that acquired our common stock through the exercise of employee stock options or otherwise as compensation;

- persons that acquired our common stock through the exercise of warrants or conversion rights under convertible instruments;
- persons that hold our common stock as "qualified small business stock" under Section 1202 of the Code or "Section 1244 stock" under Section 1244 of the Code;
- persons that acquired our common stock in a transaction subject to the gain rollover provisions of the Code (including Section 1045 of the Code);
- persons that own, or have owned, actually or constructively, more than 5% of our common stock;
- "qualified foreign pension funds" within the meaning of Section 897(l)(2) of the Code and entities all of the interests of which are held by qualified foreign pension funds; and
- persons that hold our common stock as part of a hedging or conversion transaction, straddle, a constructive sale, or any other risk reduction strategy or integrated investment.

If a partnership (or an entity or arrangement that is treated as a partnership for U.S. federal income tax purposes) holds our common stock, the U.S. federal income tax treatment of a partner in such partnership will generally depend on the status of the partner and the activities of the partnership. Partnerships that hold our common stock and the partners in such partnerships are urged to consult their tax advisors about the particular U.S. federal income tax consequences to them of holding and disposing of our common stock.

THIS DISCUSSION IS FOR INFORMATIONAL PURPOSES ONLY AND IS NOT TAX ADVICE. PROSPECTIVE INVESTORS SHOULD CONSULT THEIR TAX ADVISORS REGARDING THE PARTICULAR U.S. FEDERAL INCOME TAX CONSEQUENCES TO THEM OF ACQUIRING, OWNING, AND DISPOSING OF OUR COMMON STOCK, AS WELL AS ANY TAX CONSEQUENCES ARISING UNDER ANY STATE, LOCAL, OR NON-U.S. TAX LAWS AND ANY U.S. FEDERAL NON-INCOME TAX LAWS, OR UNDER ANY APPLICABLE INCOME TAX TREATY.

Definition of non-U.S. holder

For purposes of this discussion, a non-U.S. holder is any beneficial owner of our common stock that is not a "U.S. person" or a partnership (including any entity or arrangement treated as a partnership) for U.S. federal income tax purposes. A U.S. person is any person that, for U.S. federal income tax purposes, is or is treated as any of the following:

- an individual who is a citizen or resident of the United States;
- a corporation (or any entity treated as a corporation for U.S. federal income tax purposes) created or organized under the laws of the United States, any state thereof, or the District of Columbia;
- an estate, the income of which is subject to U.S. federal income tax regardless of its source; or
- a trust (1) whose administration is subject to the primary supervision of a U.S. court and which has one or more "U.S. persons" within the meaning of Section 7701(a)(30) of the Code who have the authority to control all substantial decisions of the trust or (2) that has a valid election in effect under applicable Treasury Regulations to be treated as a U.S. person.

Distributions on our common stock

As described in the section titled "Dividend Policy" above, we have not paid and do not anticipate paying dividends in the foreseeable future. However, if we make cash or other property distributions on our common stock, such distributions 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. Amounts that exceed such current and accumulated earnings and profits and, therefore, are not treated as dividends for U.S. federal income tax purposes will constitute a return of capital and first be applied against and reduce the non-U.S. holder's tax basis in our common stock, but not below zero. Any amount distributed in excess of tax basis will be treated as gain realized on the sale or other disposition of our common stock and, therefore, treated as described in the section titled "— Gain on Disposition of Our Common Stock" below.

Subject to the discussions below regarding effectively connected income, backup withholding, and Sections 1471 through 1474 of the Code, or commonly referred to as the Foreign Account Tax Compliance Act (FATCA), dividends paid to a non-U.S. holder generally will be subject to U.S. federal withholding tax at a rate of 30% (or such lower rate specified by an applicable income tax treaty) of the gross amount of the dividends. To receive the benefit of a lower treaty rate, a non-U.S. holder must furnish the applicable withholding agent with a valid IRS Form W-8BEN or IRS Form W-8BEN-E (or applicable successor form) certifying such non-U.S. holder's qualification for such rate. This certification must be provided to the applicable withholding agent before the payment of dividends and updated periodically. If the non-U.S. holder holds our common stock through a financial institution or other agent acting on the non-U.S. holder's behalf, the non-U.S. holder will be required to provide appropriate documentation to the financial institution or agent, which then will be required to provide certification to the applicable withholding agent, either directly or through other intermediaries.

Non-U.S. holders that do not provide the required certification on a timely basis, but that qualify for a reduced treaty rate, may obtain a refund of any excess amounts withheld by timely filing an appropriate claim for refund with the IRS.

If a non-U.S. holder holds our common stock in connection with the conduct of a trade or business in the United States, and dividends paid on our common stock are effectively connected with such non-U.S. holder's U.S. trade or business (and are attributable to such non-U.S. holder's permanent establishment in the United States, if required by an applicable tax treaty), the non-U.S. holder generally will be exempt from U.S. federal withholding tax. To claim the exemption, the non-U.S. holder must generally furnish a valid IRS Form W-8ECI (or applicable successor form) to the applicable withholding agent.

However, any such effectively connected dividends paid on our common stock generally will be subject to U.S. federal income tax on a net income basis at regular U.S. federal income tax rates in the same manner as if the non-U.S. holder were a resident of the United States. A non-U.S. holder that is a foreign corporation also may be subject to an additional branch profits tax equal to 30% (or such lower rate specified by an applicable income tax treaty) of its effectively connected earnings and profits for the taxable year, as adjusted for certain items. Non-U.S. holders are urged to consult their tax advisors about any applicable income tax treaties that may provide for different rules.

Gain on disposition of our common stock

Subject to the discussions below regarding backup withholding and FATCA, a non-U.S. holder generally will not be subject to U.S. federal income tax on any gain realized on the sale or other disposition of our common stock, unless:

- the gain is effectively connected with the non-U.S. holder's conduct of a trade or business in the United States and, if required by an applicable income tax treaty, is attributable to the non-U.S. holder's permanent establishment in the United States;
- the non-U.S. holder is a nonresident alien individual present in the United States for 183 days or more during the taxable year of the sale or other disposition, and certain other requirements 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 USRPHC, for U.S. federal income tax purposes at any time within the shorter of the five-year period preceding the sale or other disposition or the non-U.S. holder’s holding period for our common stock, and our common stock is not regularly traded on an established securities market as defined by applicable Treasury Regulations.

Determining 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 other trade or business assets and our foreign real property interests. We do not believe that we are, or have been, and do not anticipate becoming, a USRPHC for U.S. federal income tax purposes, although there can be no assurance that we will not in the future become a USRPHC. Even if we are or were to become a USRPHC, gain arising from the sale or other disposition by a non-U.S. holder of our common stock may not be subject to U.S. federal income tax if our common stock is “regularly traded,” as defined by applicable Treasury Regulations, on an established securities market.

Gain described in the first bullet point above generally will be subject to U.S. federal income tax on a net income basis at regular U.S. federal income tax rates in the same manner as if the non-U.S. holder were a resident of the United States. A non-U.S. holder that is a foreign corporation also may be subject to an additional branch profits tax equal to 30% (or such lower rate specified by an applicable income tax treaty) of its effectively connected earnings and profits for the taxable year, as adjusted for certain items. Gain described in the second bullet point above will be subject to U.S. federal income tax at a flat 30% rate (or such lower rate specified by an applicable income tax treaty) but may be offset by certain U.S.-source capital losses (even though the individual is not considered a resident of the United States), provided that the non-U.S. holder has timely filed U.S. federal income tax returns with respect to such losses. Non-U.S. holders are urged to consult their tax advisors about any applicable income tax treaties that may provide for different rules.

Information reporting and backup withholding

Annual reports are required to be filed with the IRS and provided to each non-U.S. holder indicating the amount of distributions on our common stock paid to such non-U.S. holder and the amount of any tax withheld with respect to those distributions. These information reporting requirements apply even if no withholding was required (because the distributions were effectively connected with the non-U.S. holder’s conduct of a U.S. trade or business, or withholding was reduced or eliminated by an applicable income tax treaty) and regardless of whether such distributions constitute dividends. This information also may be made available under a specific treaty or agreement with the tax authorities in the country in which the non-U.S. holder resides or is established. Backup withholding, currently at a 24% rate, generally will not apply to payments to a non-U.S. holder of dividends on or the gross proceeds of a sale or other disposition of our common stock provided that the non-U.S. holder furnishes the required certification for its non-U.S. status, such as by providing a valid IRS Form W-8BEN, IRS Form W-8BEN-E, or IRS Form W-8ECI, or certain other requirements are met. Backup withholding may apply if the payor has actual knowledge, or reason to know, that the non-U.S. holder is a U.S. person who is not an exempt recipient.

Backup withholding is not an additional tax. If any amount is withheld under the backup withholding rules, non-U.S. holders are urged to consult their tax advisors about the possibility of and procedure for obtaining a refund or a credit against the non-U.S. holder’s U.S. federal income tax liability, if any.

Foreign account tax compliance act

FATCA imposes a U.S. federal withholding tax of 30% on certain payments made to a “foreign financial institution” (as specially defined under the FATCA rules) unless such institution enters into an agreement with

the U.S. government to withhold on certain payments and to collect and provide to the U.S. tax authorities substantial information regarding certain 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 foreign entities with U.S. owners) or an exemption applies. FATCA also generally will impose a U.S. federal withholding tax of 30% on certain payments made to a non-financial foreign entity unless such entity provides the withholding agent a certification identifying certain direct and indirect U.S. owners of the entity or an exemption applies. An intergovernmental agreement between the United States and an applicable non-U.S. country may modify these requirements. Under certain circumstances, a non-U.S. holder might be eligible for refunds or credits of such taxes. FATCA applies to dividends paid on our common stock and, subject to the proposed Treasury Regulations described below, also applies to gross proceeds from sales or other dispositions of our common stock. The U.S. Treasury Department released proposed Treasury Regulations which, if finalized in their present form, would eliminate the U.S. federal withholding tax of 30% applicable to the gross proceeds of a sale or other disposition of our common stock. In its preamble to such proposed Treasury Regulations, the U.S. Treasury Department stated that taxpayers may generally rely on the proposed Treasury Regulations until final regulations are issued.

Prospective investors are urged to consult with their tax advisors about the possible implications of FATCA on their investment in our common stock.

Underwriting

We are offering the shares of common stock described in this prospectus through a number of underwriters. J.P. Morgan Securities LLC, SVB Securities LLC, Stifel, Nicolaus & Company, Incorporated and RBC Capital Markets, LLC are acting as joint book-running managers of the offering and as representatives of the underwriters. We have entered into an underwriting agreement with the underwriters. Subject to the terms and conditions of the underwriting agreement, we have agreed to sell to the underwriters, and each underwriter has severally agreed to purchase, at the public offering price less the underwriting discounts and commissions set forth on the cover page of this prospectus, the number of shares of common stock listed next to its name in the following table:

Name	Number of Shares
J.P. Morgan Securities LLC	
SVB Securities LLC	
Stifel, Nicolaus & Company, Incorporated	
RBC Capital Markets, LLC	
Chardan Capital Markets, LLC	
Total	

The underwriters are committed to purchase all the common stock offered by us if they purchase any shares. The underwriting agreement also provides that if an underwriter defaults, the purchase commitments of non-defaulting underwriters may also be increased or the offering may be terminated.

The underwriters propose to offer the common stock directly to the public at the initial public offering price set forth on the cover page of this prospectus and to certain dealers at that price less a concession not in excess of $ per share. Any such dealers may resell shares to certain other brokers or dealers at a discount of up to $ per share from the initial public offering price. After the initial offering of the shares to the public, if all of the common stock is not sold at the initial public offering price, the underwriters may change the offering price and the other selling terms. Sales of any shares made outside of the United States may be made by affiliates of the underwriters.

The underwriters have an option to buy up to additional shares of common stock from us to cover sales of shares by the underwriters which exceed the number of shares specified in the table above. The underwriters have 30 days from the date of this prospectus to exercise this option to purchase additional shares. If any shares are purchased with this option to purchase additional shares, the underwriters will purchase shares in approximately the same proportion as shown in the table above. If any additional shares of common stock are purchased, the underwriters will offer the additional shares on the same terms as those on which the shares are being offered.

The underwriting fee is equal to the public offering price per share of common stock less the amount paid by the underwriters to us per share of common stock. The underwriting fee is $ per share. The following table shows the per share and total underwriting discounts and commissions to be paid to the underwriters assuming both no exercise and full exercise of the underwriters' option to purchase additional shares.

	Without Option to Purchase Additional Shares Exercise	With Full Option to Purchase Additional Shares Exercise
Per Share	$	$
Total	$	$

We estimate that the total expenses of this offering, including registration, filing and listing fees, printing fees and legal and accounting expenses, but excluding the underwriting discounts and commissions, will be approximately $.

A prospectus in electronic format may be made available on the web sites maintained by one or more underwriters, or selling group members, if any, participating in the offering. The underwriters may agree to allocate a number of shares to underwriters and selling group members for sale to their online brokerage account holders. Internet distributions will be allocated by the representatives to underwriters and selling group members that may make Internet distributions on the same basis as other allocations.

We have agreed that we will not (i) offer, pledge, 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, lend or otherwise transfer or dispose of, directly or indirectly, or submit to, or file with, the Securities and Exchange Commission a registration statement under the Securities Act relating to, any shares of our common stock or securities convertible into or exercisable or exchangeable for any shares of our common stock, or publicly disclose the intention to make any offer, sale, pledge, loan, disposition or filing, or (ii) enter into any swap or other arrangement that transfers all or a portion of the economic consequences associated with the ownership of any shares of common stock or any such other securities (regardless of whether any of these transactions are to be settled by the delivery of shares of common stock or such other securities, in cash or otherwise), in each case without the prior written consent of J.P. Morgan Securities LLC and SVB Securities LLC for a period of 180 days after the date of this prospectus, other than the shares of our common stock to be sold in this offering. The restrictions on our actions, as described above, do not apply to certain transactions, including .

Our directors and executive officers, and substantially all of our shareholders (such persons, the “lock-up parties”) have entered into lock up agreements with the underwriters prior to the commencement of this offering pursuant to which each lock-up party, with limited exceptions, for a period of 180 days after the date of this prospectus (such period, the “restricted period”), may not (and may not cause any of their direct or indirect affiliates to), without the prior written consent of J.P. Morgan Securities LLC and SVB Securities LLC, (1) offer, pledge, 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, lend or otherwise transfer or dispose of, directly or indirectly, any shares of our common stock or any securities convertible into or exercisable or exchangeable for our common stock (including, without limitation, common stock or such other securities which may be deemed to be beneficially owned by such lock-up parties in accordance with the rules and regulations of the SEC and securities which may be issued upon exercise of a stock option or warrant (collectively with the common stock, the “lock-up securities”)), (2) enter into any hedging, swap or other agreement or transaction that transfers, in whole or in part, any of the economic consequences of ownership of the lock-up securities, whether any such transaction described in clause (1) or (2) above is to be settled by delivery of lock-up securities, in cash or otherwise, (3) make any demand for, or exercise any right with respect to, the registration of any lock-up securities, or (4) publicly disclose the intention to do any of the foregoing. Such persons or entities have further acknowledged that these undertakings preclude them from engaging in any hedging or other transactions or arrangements (including, without limitation, any short sale or the purchase or sale of, or entry into, any put or call option, or combination thereof, forward, swap or any other derivative transaction or instrument, however described or defined) designed or intended, or which could reasonably be expected to lead to or result in, a sale or disposition or transfer (by any person or entity, whether or not a signatory to such agreement) of any economic consequences of ownership, in whole or in part, directly or indirectly, of any lock-up securities, whether any such transaction or arrangement (or instrument provided for thereunder) would be settled by delivery of lock-up securities, in cash or otherwise.

The restrictions described in the immediately preceding paragraph and contained in the lock-up agreements between the underwriters and the lock-up parties do not apply, subject in certain cases to various conditions, to certain transactions, including (a) transfers of lock-up securities: (i) as bona fide gifts, or for bona fide estate planning purposes; (ii) by will or intestacy; (iii) to any trust for the direct or indirect benefit of the lock-up party or any immediate family member, or if the lock-up party is a trust, to a trustor, trustee (or co-trustee) or beneficiary of the trust or to the estate of a beneficiary of the trust; (iv) to a partnership, limited liability company

or other entity of which the lock-up party and its immediate family members are the legal and beneficial owner of all of the outstanding equity securities or similar interests; (v) to a nominee or custodian of a person or entity to whom a disposition or transfer would be permissible under clauses (i) through (iv); (vi) in the case of a corporation, partnership, limited liability company, trust or other business entity, (A) to another corporation, partnership, limited liability company, trust or other business entity that is an affiliate of the lock-up party, or to any investment fund or other entity controlling, controlled by, managing or managed by or under common control with the lock-up party or its affiliates or (B) as part of a distribution, transfer or disposition to stockholders, partners, members, beneficiaries or other equity holders of the lock-up party; (vii) by operation of law; (viii) to us from an employee upon death, disability or termination of employment of such employee; (ix) as part of a sale of lock-up securities acquired in this offering or in open market transactions after the completion of this offering; (x) to us in connection with the vesting, settlement or exercise of restricted stock units, options, warrants or other rights to purchase shares of our common stock (including "net" or "cashless" exercise), including for the payment of exercise price and tax and remittance payments; (xi) pursuant to a bona fide third-party tender offer, merger, consolidation or other similar transaction approved by our board of directors and made to all shareholders involving a change in control, provided that if such transaction is not completed, all such lock-up securities would remain subject to the restrictions in the immediately preceding paragraph; or (xii) to an immediate family member of the lock-up party; (b) exercise of the options, settlement of RSUs or other equity awards, or the exercise of warrants granted pursuant to plans described in in this prospectus, provided that any lock-up securities received upon such exercise, vesting or settlement would be subject to restrictions similar to those in the immediately preceding paragraph; (c) the conversion of outstanding preferred stock, warrants to acquire preferred stock, or convertible securities into shares of our common stock or warrants to acquire shares of our common stock, provided that any common stock or warrant received upon such conversion would be subject to restrictions similar to those in the immediately preceding paragraph; and (d) the establishment by lock-up parties of trading plans under Rule 10b5-1 under the Exchange Act, provided that such plan does not provide for the transfer of lock-up securities during the restricted period.

J.P. Morgan Securities LLC and SVB Securities LLC, in their sole discretion, may release the securities subject to any of the lock-up agreements with the underwriters described above, in whole or in part at any time.

We have agreed to indemnify the underwriters against certain liabilities, including liabilities under the Securities Act.

We intend to apply to have our common stock approved for listing on the Nasdaq Global Market under the symbol "LXEO".

In connection with this offering, the underwriters may engage in stabilizing transactions, which involves making bids for, purchasing and selling shares of common stock in the open market for the purpose of preventing or retarding a decline in the market price of the common stock while this offering is in progress. These stabilizing transactions may include making short sales of common stock, which involves the sale by the underwriters of a greater number of shares of common stock than they are required to purchase in this offering, and purchasing shares of common stock on the open market to cover positions created by short sales. Short sales may be "covered" shorts, which are short positions in an amount not greater than the underwriters' option to purchase additional shares referred to above, or may be "naked" shorts, which are short positions in excess of that amount. The underwriters may close out any covered short position either by exercising their option to purchase additional shares, in whole or in part, or by purchasing shares in the open market. In making this determination, the underwriters will consider, among other things, the price of shares available for purchase in the open market compared to the price at which the underwriters may purchase shares through the option to purchase additional shares. A naked short position is more likely to be created if the underwriters are concerned that there may be downward pressure on the price of the common stock in the open market that could adversely affect investors who purchase in this offering. To the extent that the underwriters create a naked short position, they will purchase shares in the open market to cover the position.

The underwriters have advised us that, pursuant to Regulation M of the Securities Act, they may also engage in other activities that stabilize, maintain or otherwise affect the price of the common stock, including the imposition of penalty bids. This means that if the representatives of the underwriters purchase common stock in the open market in stabilizing transactions or to cover short sales, the representatives can require the underwriters that sold those shares as part of this offering to repay the underwriting discount received by them.

These activities may have the effect of raising or maintaining the market price of the common stock or preventing or retarding a decline in the market price of the common stock, and, as a result, the price of the common stock may be higher than the price that otherwise might exist in the open market. If the underwriters commence these activities, they may discontinue them at any time. The underwriters may carry out these transactions on The Nasdaq Stock Market, in the over-the-counter market or otherwise.

In addition, in connection with this offering certain of the underwriters (and selling group members) may engage in passive market making transactions in our common stock on The Nasdaq Stock Market prior to the pricing and completion of this offering. Passive market making consists of displaying bids on The Nasdaq Stock Market no higher than the bid prices of independent market makers and making purchases at prices no higher than these independent bids and effected in response to order flow. Net purchases by a passive market maker on each day are generally limited to a specified percentage of the passive market maker's average daily trading volume in the common stock during a specified period and must be discontinued when such limit is reached. Passive market making may cause the price of our common stock to be higher than the price that otherwise would exist in the open market in the absence of these transactions. If passive market making is commenced, it may be discontinued at any time.

Prior to this offering, there has been no public market for our common stock. The initial public offering price will be determined by negotiations between us and the representatives of the underwriters. In determining the initial public offering price, we and the representatives of the underwriters expect to consider a number of factors including:

- the information set forth in this prospectus and otherwise available to the representatives;
- our prospects and the history and prospects for the industry in which we compete;
- an assessment of our management;
- our prospects for future earnings;
- the general condition of the securities markets at the time of this offering;
- the recent market prices of, and demand for, publicly traded common stock of generally comparable companies; and
- other factors deemed relevant by the underwriters and us.

Neither we nor the underwriters can assure investors that an active trading market will develop for our common stock, or that the shares will trade in the public market at or above the initial public offering price.

Other than in the United States, no action has been taken by us or the underwriters that would permit a public offering of the securities offered by this prospectus in any jurisdiction where action for that purpose is required. The securities offered by this prospectus may not be offered or sold, directly or indirectly, nor may this prospectus or any other offering material or advertisements in connection with the offer and sale of any such securities be distributed or published in any jurisdiction, except under circumstances that will result in compliance with the applicable rules and regulations of that jurisdiction. Persons into whose possession this prospectus comes are advised to inform themselves about and to observe any restrictions relating to the offering and the distribution of

this prospectus. This prospectus does not constitute an offer to sell or a solicitation of an offer to buy any securities offered by this prospectus in any jurisdiction in which such an offer or a solicitation is unlawful.

Certain of the underwriters and their affiliates have provided in the past to us and our affiliates and may provide from time to time in the future certain commercial banking, financial advisory, investment banking and other services for us and such affiliates in the ordinary course of their business, for which they have received and may continue to receive customary fees and commissions. In addition, from time to time, certain of the underwriters and their affiliates may effect transactions for their own account or the account of customers, and hold on behalf of themselves or their customers, long or short positions in our debt or equity securities or loans, and may do so in the future.

Notice to prospective investors in the european economic area

In relation to each Member State of the European Economic Area (each a "Relevant State"), no shares of our common stock have been offered or will be offered pursuant to the offering to the public in that Relevant State prior to the publication of a prospectus in relation to the shares of our common stock which has been approved by the competent authority in that Relevant State or, where appropriate, approved in another Relevant State and notified to the competent authority in that Relevant State, all in accordance with the Prospectus Regulation), except that offers of Shares may be made to the public in that Relevant State at any time under the following exemptions under the Prospectus Regulation:

a) to any legal entity which is a qualified investor as defined under the Prospectus Regulation;

b) to fewer than 150 natural or legal persons (other than qualified investors as defined under the Prospectus Regulation), subject to obtaining the prior consent of the underwriters for any such offer; or

c) in any other circumstances falling within Article 1(4) of the Prospectus Regulation,

provided that no such offer of shares of common stock shall require the Issuer or any underwriter to publish a prospectus pursuant to Article 3 of the Prospectus Regulation or supplement a prospectus pursuant to Article 23 of the Prospectus Regulation.

Each person in a Relevant State who initially acquires any shares of common stock or to whom any offer is made will be deemed to have represented, acknowledged and agreed to and with the Company and the underwriters that it is a qualified investor within the meaning of the Prospectus Regulation.

In the case of any shares of common stock being offered to a financial intermediary as that term is used in Article 5(1) of the Prospectus Regulation, each such financial intermediary will be deemed to have represented, acknowledged and agreed that the shares of common stock acquired by it in the offer have not been acquired on a non-discretionary basis on behalf of, nor have they been acquired with a view to their offer or resale to, persons in circumstances which may give rise to an offer to the public other than their offer or resale in a Relevant State to qualified investors, in circumstances in which the prior consent of the underwriters has been obtained to each such proposed offer or resale.

The Company, the underwriters and their affiliates will rely upon the truth and accuracy of the foregoing representations, acknowledgements and agreements.

For the purposes of this provision, the expression an "offer to the public" in relation to any shares of common stock in any Relevant State means the communication in any form and by any means of sufficient information on the terms of the offer and any Shares to be offered so as to enable an investor to decide to purchase or subscribe for any Shares, and the expression "Prospectus Regulation" means Regulation (EU) 2017/1129.

Notice to prospective investors in the United Kingdom

In relation to the United Kingdom, or the UK, no shares of our common stock have been offered or will be offered pursuant to the offering to the public in the UK prior to the publication of a prospectus in relation to the shares of common stock which has been approved by the Financial Conduct Authority in the UK in accordance with the UK Prospectus Regulation and the Financial Services and Markets Act 2000, or the FSMA, except that offers of shares of common stock may be made to the public in the UK at any time under the following exemptions under the UK Prospectus Regulation and the FSMA:

a) to any legal entity which is a qualified investor as defined under the UK Prospectus Regulation;

b) to fewer than 150 natural or legal persons (other than qualified investors as defined under the UK Prospectus Regulation), subject to obtaining the prior consent of the underwriters for any such offer; or

c) at any time in other circumstances falling within section 86 of the FSMA,

provided that no such offer of shares of common stock shall require the issuer or any underwriter to publish a prospectus pursuant to Section 85 of the FSMA or Article 3 of the UK Prospectus Regulation or supplement a prospectus pursuant to Article 23 of the UK Prospectus Regulation.

Each person in the UK who initially acquires any shares of common stock or to whom any offer is made will be deemed to have represented, acknowledged and agreed to and with the Company and the underwriters that it is a qualified investor within the meaning of the UK Prospectus Regulation.

In the case of any shares of common stock being offered to a financial intermediary as that term is used in Article 5(1) of the UK Prospectus Regulation, each such financial intermediary will be deemed to have represented, acknowledged and agreed that the Shares acquired by it in the offer have not been acquired on a non-discretionary basis on behalf of, nor have they been acquired with a view to their offer or resale to, persons in circumstances which may give rise to an offer to the public other than their offer or resale in the UK to qualified investors, in circumstances in which the prior consent of the underwriters has been obtained to each such proposed offer or resale.

The Company, the underwriters and their affiliates will rely upon the truth and accuracy of the foregoing representations, acknowledgements and agreements.

For the purposes of this provision, the expression an “offer to the public” in relation to any shares of our common stock in the UK means the communication in any form and by any means of sufficient information on the terms of the offer and any shares of our common stock to be offered so as to enable an investor to decide to purchase or subscribe for any shares of our common stock, the expression “UK Prospectus Regulation” means Regulation (EU) 2017/1129 as it forms part of domestic law by virtue of the European Union (Withdrawal) Act 2018, and the expression “FSMA” means the Financial Services and Markets Act 2000.

This document is for distribution only to persons who (i) have professional experience in matters relating to investments and who qualify as investment professionals within the meaning of Article 19(5) of the Financial Services and Markets Act 2000 (Financial Promotion) Order 2005 (as amended), or the “Financial Promotion Order,” (ii) are persons falling within Article 49(2)(a) to (d) (“high net worth companies, unincorporated associations etc.”) of the Financial Promotion Order, (iii) are outside the United Kingdom, or (iv) are persons to whom an invitation or inducement to engage in investment activity (within the meaning of Section 21 of the FSMA) in connection with the issue or sale of any securities may otherwise lawfully be communicated or caused to be communicated (all such persons together being referred to as “relevant persons”). This document is directed only at relevant persons and must not be acted on or relied on by persons who are not relevant persons. Any investment or investment activity to which this document relates is available only to relevant persons and will be engaged in only with relevant persons.

Notice to prospective investors in Canada

The shares of common stock may be sold only to purchasers purchasing, or deemed to be purchasing, as principal that are accredited investors, as defined in National Instrument 45-106 Prospectus Exemptions or subsection 73.3(1) of the Securities Act (Ontario), and are permitted clients, as defined in National Instrument 31-103 Registration Requirements, Exemptions and Ongoing Registrant Obligations. Any resale of the shares of common stock must be made in accordance with an exemption from, or in a transaction not subject to, the prospectus requirements of applicable securities laws.

Securities legislation in certain provinces or territories of Canada may provide a purchaser with remedies for rescission or damages if this prospectus (including any amendment thereto) contains a misrepresentation, provided that the remedies for rescission or damages are exercised by the purchaser within the time limit prescribed by the securities legislation of the purchaser's province or territory. The purchaser should refer to any applicable provisions of the securities legislation of the purchaser's province or territory for particulars of these rights or consult with a legal advisor.

Pursuant to section 3A.3 of National Instrument 33-105 Underwriting Conflicts (NI 33-105), the underwriters are not required to comply with the disclosure requirements of NI 33-105 regarding underwriter conflicts of interest in connection with this offering.

Notice to prospective investors in Switzerland

The shares of common stock may not be publicly offered in Switzerland and will not be listed on the SIX Swiss Exchange, or SIX, or on any other stock exchange or regulated trading facility in Switzerland. This document does not constitute a prospectus within the meaning of, and has been prepared without regard to the disclosure standards for issuance prospectuses under art. 652a or art. 1156 of the Swiss Code of Obligations or the disclosure standards for listing prospectuses under art. 27 ff. of the SIX Listing Rules or the listing rules of any other stock exchange or regulated trading facility in Switzerland. Neither this document nor any other offering or marketing material relating to the shares of common stock or the offering may be publicly distributed or otherwise made publicly available in Switzerland.

Neither this document nor any other offering or marketing material relating to the offering, the Company, the shares of common stock have been or will be filed with or approved by any Swiss regulatory authority. In particular, this document will not be filed with, and the offer of shares of common stock will not be supervised by, the Swiss Financial Market Supervisory Authority FINMA, or FINMA, and the offer of shares of common stock has not been and will not be authorized under the Swiss Federal Act on Collective Investment Schemes, or CISA. The investor protection afforded to acquirers of interests in collective investment schemes under the CISA does not extend to acquirers of shares of common stock.

Notice to prospective investors in Hong Kong

The shares of common stock have not been offered or sold and will not be offered or sold in Hong Kong, by means of any document, other than (a) to "professional investors" as defined in the Securities and Futures Ordinance (Cap. 571 of the laws of Hong Kong), or SFO, of Hong Kong and any rules made thereunder; or (b) in other circumstances which do not result in the document being a "prospectus" as defined in the Companies (Winding Up and Miscellaneous Provisions) Ordinance (Cap. 32) of the laws of Hong Kong), or CO, or which do not constitute an offer to the public within the meaning of the CO. No advertisement, invitation or document relating to the shares of common stock has been or may be issued or has been or may be in the possession of any person for the purposes of issue, whether in Hong Kong or elsewhere, which is directed at, or the contents of which are likely to be accessed or read by, the public of Hong Kong (except if permitted to do so under the securities laws of Hong Kong) other

than with respect to shares of common stock which are or are intended to be disposed of only to persons outside Hong Kong or only to "professional investors" as defined in the SFO and any rules made thereunder.

Notice to prospective investors in Singapore

Each joint book-running manager has acknowledged that this prospectus has not been registered as a prospectus with the Monetary Authority of Singapore. Accordingly, each joint book-running manager has represented and agreed that it has not offered or sold any shares of common stock or caused the shares of common stock to be made the subject of an invitation for subscription or purchase and will not offer or sell any shares of common stock or cause the shares of common stock to be made the subject of an invitation for subscription or purchase, and has not circulated or distributed, nor will it circulate or distribute, this prospectus or any other document or material in connection with the offer or sale, or invitation for subscription or purchase, of the shares of common stock, whether directly or indirectly, to any person in Singapore other than:

(a) to an institutional investor (as defined in Section 4A of the Securities and Futures Act (Chapter 289) of Singapore, as modified or amended from time to time, or the SFA) pursuant to Section 274 of the SFA;

(b) to a relevant person (as defined in Section 275(2) of the SFA) pursuant to Section 275(1) of the SFA, or any person pursuant to Section 275(1A) of the SFA, and in accordance with the conditions specified in Section 275 of the SFA; or

(c) otherwise pursuant to, and in accordance with the conditions of, any other applicable provision of the SFA.

Where the shares of common stock are subscribed or purchased under Section 275 of the SFA by a relevant person which is:

(a) a corporation (which is not an accredited investor (as defined in Section 4A of the SFA)) the sole business of which is to hold investments and the entire share capital of which is owned by one or more individuals, each of whom is an accredited investor; or

(b) a trust (where the trustee is not an accredited investor) whose sole purpose is to hold investments and each beneficiary of the trust is an individual who is an accredited investor, securities or securities-based derivatives contracts (each term as defined in Section 2(1) of the SFA) of that corporation or the beneficiaries' rights and interest (howsoever described) in that trust shall not be transferred within six months after that corporation or that trust has acquired the shares of common stock pursuant to an offer made under Section 275 of the SFA except:

    (i) to an institutional investor or to a relevant person, or to any person arising from an offer referred to in Section 275(1A) or Section 276(4)(i)(B) of the SFA;

    (ii) where no consideration is or will be given for the transfer;

    (iii) where the transfer is by operation of law;

    (iv) as specified in Section 276(7) of the SFA; or

    (v) as specified in Regulation 37A of the Securities and Futures (Offers of Investments) (Securities and Securities-based Derivatives Contracts) Regulations 2018.

Singapore SFA Product Classification—In connection with Section 309B of the SFA and the CMP Regulations 2018, unless otherwise specified before an offer of shares of common stock, we have determined, and hereby notify all relevant persons (as defined in Section 309A(1) of the SFA), that the shares of common stock are ''prescribed capital markets products'' (as defined in the CMP Regulations 2018) and Excluded Investment Products (as defined in MAS Notice SFA 04-N12: Notice on the Sale of Investment Products and MAS Notice FAA-N16: Notice on Recommendations on Investment Products).

Notice to prospective investors in Japan

The shares of common stock have not been and will not be registered pursuant to Article 4, Paragraph 1 of the Financial Instruments and Exchange Act. Accordingly, none of the shares of common stock nor any interest therein may be offered or sold, directly or indirectly, in Japan or to, or for the benefit of, any "resident" of Japan (which term as used herein means any person resident in Japan, including any corporation or other entity organized under the laws of Japan), or to others for re-offering or resale, directly or indirectly, in Japan or to or for the benefit of a resident of Japan, except pursuant to an exemption from the registration requirements of, and otherwise in compliance with, the Financial Instruments and Exchange Act and any other applicable laws, regulations and ministerial guidelines of Japan in effect at the relevant time.

Notice to prospective investors in the United Arab Emirates

The shares of common stock have not been, and are not being, publicly offered, sold, promoted or advertised in the United Arab Emirates (including the Dubai International Financial Centre, or the DIFC) other than in compliance with the laws of the United Arab Emirates (and the DIFC) governing the issue, offering and sale of securities. Further, this prospectus does not constitute a public offer of securities in the United Arab Emirates (including the DIFC) and is not intended to be a public offer. This prospectus has not been approved by or filed with the Central Bank of the United Arab Emirates, the Securities and Commodities Authority or the Dubai Financial Services Authority, or the DFSA.

Notice to prospective investors in Israel

In the State of Israel this prospectus shall not be regarded as an offer to the public to purchase shares of common stock under the Israeli Securities Law, 5728—1968, which requires a prospectus to be published and authorized by the Israel Securities Authority, if it complies with certain provisions of Section 15 of the Israeli Securities Law, 5728–1968, including, inter alia, if: (i) the offer is made, distributed or directed to not more than 35 investors, subject to certain conditions, or the Addressed Investors; or (ii) the offer is made, distributed or directed to certain qualified investors defined in the First Addendum of the Israeli Securities Law, 5728—1968, subject to certain conditions, or the "Qualified Investors". The Qualified Investors shall not be taken into account in the count of the Addressed Investors and may be offered to purchase securities in addition to the 35 Addressed Investors. We have not and will not take any action that would require it to publish a prospectus in accordance with and subject to the Israeli Securities Law, 5728—1968. We have not and will not distribute this prospectus or make, distribute or direct an offer to subscribe for our shares of common stock to any person within the State of Israel, other than to Qualified Investors and up to 35 Addressed Investors.

Qualified Investors may have to submit written evidence that they meet the definitions set out in of the First Addendum to the Israeli Securities Law, 5728—1968. In particular, we may request, as a condition to be offered shares of common stock, that Qualified Investors will each represent, warrant and certify to us and/or to anyone acting on our behalf: (i) that it is an investor falling within one of the categories listed in the First Addendum to the Israeli Securities Law, 5728—1968; (ii) which of the categories listed in the First Addendum to the Israeli Securities Law, 5728—1968 regarding Qualified Investors is applicable to it; (iii) that it will abide by all provisions set forth in the Israeli Securities Law, 5728—1968 and the regulations promulgated thereunder in connection with the offer to be issued shares of common stock; (iv) that the shares of common stock that it will be issued are, subject to exemptions available under the Israeli Securities Law, 5728—1968: (a) for its own account; (b) for investment purposes only; and (c) not issued with a view to resale within the State of Israel, other than in accordance with the provisions of the Israeli Securities Law, 5728—1968; and (v) that it is willing to provide further evidence of its Qualified Investor status. Addressed Investors may have to submit written evidence in respect of their identity and may have to sign and submit a declaration containing, inter alia, the Addressed Investor's name, address and passport number or Israeli identification number.

Notice to prospective investors in Australia

This prospectus:

(a) does not constitute a disclosure document or a prospectus under Chapter 6D.2 of the Corporations Act 2001 (Cth), or the Corporations Act;

(b) has not been, and will not be, lodged with the Australian Securities and Investments Commission, or ASIC, as a disclosure document for the purposes of the Corporations Act and does not purport to include the information required of a disclosure document for the purposes of the Corporations Act; and

(c) may only be provided in Australia to select investors who are able to demonstrate that they fall within one or more of the categories of investors, available under section 708 of the Corporations Act, or Exempt Investors.

The shares of common stock may not be directly or indirectly offered for subscription or purchased or sold, and no invitations to subscribe for or buy the shares of common stock may be issued, and no draft or definitive offering memorandum, advertisement or other offering material relating to any shares of common stock may be distributed in Australia, except where disclosure to investors is not required under Chapter 6D of the Corporations Act or is otherwise in compliance with all applicable Australian laws and regulations. By submitting an application for the shares of common stock, you represent and warrant to us that you are an Exempt Investor.

As any offer of shares of common stock under this document will be made without disclosure in Australia under Chapter 6D.2 of the Corporations Act, the offer of those securities for resale in Australia within 12 months may, under section 707 of the Corporations Act, require disclosure to investors under Chapter 6D.2 if none of the exemptions in section 708 applies to that resale. By applying for the shares of common stock you undertake to us that you will not, for a period of 12 months from the date of issue of the shares of common stock, offer, transfer, assign or otherwise alienate those shares of common stock to investors in Australia except in circumstances where disclosure to investors is not required under Chapter 6D.2 of the Corporations Act or where a compliant disclosure document is prepared and lodged with ASIC.

Notice to prospective investors in China

This prospectus will not be circulated or distributed in the PRC and the shares of common stock will not be offered or sold, and will not be offered or sold to any person for re-offering or resale directly or indirectly to any residents of the PRC except pursuant to any applicable laws and regulations of the PRC. Neither this prospectus nor any advertisement or other offering material may be distributed or published in the PRC, except under circumstances that will result in compliance with applicable laws and regulations.

Notice to prospective investors in Korea

The shares of common stock have not been and will not be registered under the Financial Investments Services and Capital Markets Act of Korea and the decrees and regulations thereunder, or the FSCMA, and the shares of common stock have been and will be offered in Korea as a private placement under the FSCMA. None of the shares of common stock may be offered, sold or delivered directly or indirectly, or offered or sold to any person for re-offering or resale, directly or indirectly, in Korea or to any resident of Korea except pursuant to the applicable laws and regulations of Korea, including the FSCMA and the Foreign Exchange Transaction Law of Korea and the decrees and regulations thereunder, or FETL. Furthermore, the purchaser of the shares of common stock shall comply with all applicable regulatory requirements (including but not limited to requirements under the FETL) in connection with the purchase of the shares of common stock. By the purchase of the shares of common stock, the

relevant holder thereof will be deemed to represent and warrant that if it is in Korea or is a resident of Korea, it purchased the shares of common stock pursuant to the applicable laws and regulations of Korea.

Notice to prospective investors in Saudi Arabia

This document may not be distributed in the Kingdom of Saudi Arabia except to such persons as are permitted under the Offers of Securities Regulations as issued by the board of the Saudi Arabian Capital Market Authority, or CMA, pursuant to resolution number 2-11-2004 dated 4 October 2004 as amended by resolution number 1-28-2008, as amended, or the CMA Regulations. The CMA does not make any representation as to the accuracy or completeness of this document and expressly disclaims any liability whatsoever for any loss arising from, or incurred in reliance upon, any part of this document. Prospective purchasers of the securities offered hereby should conduct their own due diligence on the accuracy of the information relating to the securities. If you do not understand the contents of this document, you should consult an authorized financial adviser.

Notice to prospective investors in the Dubai International Financial Centre

This document relates to an Exempt Offer in accordance with the Markets Rules 2012 of the DFSA. This document is intended for distribution only to persons of a type specified in the Markets Rules 2012 of the DFSA. It must not be delivered to, or relied on by, any other person. The DFSA has no responsibility for reviewing or verifying any documents in connection with Exempt Offers. The DFSA has not approved this prospectus supplement nor taken steps to verify the information set forth herein and has no responsibility for this document. The securities to which this document relates may be illiquid and/or subject to restrictions on their resale. Prospective purchasers of the securities offered should conduct their own due diligence on the securities. If you do not understand the contents of this document, you should consult an authorized financial advisor.

In relation to its use in the DIFC, this document is strictly private and confidential and is being distributed to a limited number of investors and must not be provided to any person other than the original recipient, and may not be reproduced or used for any other purpose. The interests in the securities may not be offered or sold directly or indirectly to the public in the DIFC.

Notice to prospective investors in Bermuda

Shares of common stock may be offered or sold in Bermuda only in compliance with the provisions of the Investment Business Act of 2003 of Bermuda which regulates the sale of securities in Bermuda. Additionally, non-Bermudian persons (including companies) may not carry on or engage in any trade or business in Bermuda unless such persons are permitted to do so under applicable Bermuda legislation.

Legal matters

The validity of the shares of common stock offered hereby will be passed upon for us by Cooley LLP, New York, New York. Certain legal matters will be passed upon for the underwriters by Goodwin Procter LLP, New York, New York.

Experts

The financial statements of Lexeo Therapeutics, Inc. as of and for the one-year period ended December 31, 2020 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 with respect to the shares of common stock being offered by this prospectus. This prospectus, which constitutes part of the registration statement, does not contain all of the information in the registration statement and its exhibits. For further information with respect to our company and the common stock offered by this prospectus, we refer you to the registration statement and its exhibits. Statements contained in this prospectus as to the contents of any contract or any other document referred to are not necessarily complete, and in each instance, we refer you to the copy of the contract or other document filed as an exhibit to the registration statement. Each of these statements is qualified in all respects by this reference.

You can read our SEC filings, including the registration statement, over the internet at the SEC's website at *www.sec.gov*. Upon completion of this offering, we will be subject to the information reporting requirements of the Exchange Act, and we will file reports, proxy statements and other information with the SEC. These reports, proxy statements and other information will be available at *www.sec.gov*.

We also maintain a website at *www.lexeotx.com*, at which you may access these materials free of charge as soon as reasonably practicable after they are electronically filed with, or furnished to, the SEC. The information contained in, or that can be accessed through, our website is not part of, and is not incorporated into, this prospectus, and the inclusion of our website address in this prospectus is only as an inactive textual reference.

LEXEO THERAPEUTICS, INC.

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Index to Financial Statements as of and for the One-year Period Ended December 31, 2020

Report of Independent Registered Public Accounting Firm

To the Stockholders and Board of Directors
Lexeo Therapeutics, Inc.:

Opinion on the Financial Statements

We have audited the accompanying balance sheet of Lexeo Therapeutics, Inc. (the Company) as of December 31, 2020, the related statements of operations and comprehensive loss, changes in convertible preferred stock and stockholders' deficit, and cash flows for the year ended December 31, 2020, and the related notes (collectively, the financial statements). In our opinion, the financial statements present fairly, in all material respects, the financial position of the Company as of December 31, 2020, and the results of its operations and its cash flows for the year ended December 31, 2020, in conformity with U.S. generally accepted accounting principles.

Basis for Opinion

These financial statements are the responsibility of the Company's management. Our responsibility is to express an opinion on these financial statements based on our audit. We are a public accounting firm registered with the Public Company Accounting Oversight Board (United States) (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 audit in accordance with the standards of the PCAOB and in accordance with auditing standards generally accepted in the United States of America. Those standards require that we plan and perform the audit to obtain reasonable assurance about whether the financial statements are free of material misstatement, whether due to error or fraud. Our audit included performing procedures to assess the risks of material misstatement of the 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 financial statements. Our audit also included evaluating the accounting principles used and significant estimates made by management, as well as evaluating the overall presentation of the financial statements. We believe that our audit provides a reasonable basis for our opinion.

/s/ KPMG LLP

We have served as the Company's auditor since 2021.

New York, New York
September 8, 2021

Lexeo Therapeutics, Inc.
Balance sheet

	As of December 31, 2020
Assets	
Current assets:	
Cash	$ 29,373,209
Prepaid expenses	33,572
Total current assets	29,406,781
Other assets	2,500
Total assets	$ 29,409,281
Liabilities, convertible preferred stock & stockholders' deficit	
Current liabilities:	
Accounts payable	$ 44,319
Taxes payable	38,304
Issuance costs payable	223,587
Deferred income	140,385
Accrued expenses and other current liabilities	251,128
Total current liabilities	697,722
Non-current liabilities:	
Preferred tranche obligation	3,010,000
Total liabilities	$ 3,707,722
Commitments and contingencies (Note 8)	
Convertible preferred stock:	
Series A convertible preferred stock, $0.0001 par value; 85,500,000 shares authorized as of December 31, 2020; 34,495,725 shares issued and outstanding as of December 31, 2020; aggregate liquidation value of $34,495,725 as of December 31, 2020	31,237,986
Stockholders' deficit:	
Common stock, $0.0001 par value, 120,000,000 shares authorized at December 31, 2020; 16,040,884 shares issued and outstanding at December 31, 2020	1,604
Additional paid-in capital	9,794
Accumulated deficit	(5,547,825)
Total stockholders' deficit	(5,536,427)
Total liabilities, convertible preferred stock, and stockholders' deficit	$ 29,409,281

The accompanying notes are an integral part of these financial statements.

Lexeo Therapeutics, Inc.
Statement of operations and comprehensive loss
For the year ended December 31, 2020

	2020
Grant revenue	$ 518,476
Operating expenses:	
Research and development	4,318,765
General and administrative	787,499
Total operating expenses	5,106,264
Loss from operations	(4,587,788)
Other expense:	
Loss on extinguishment of notes	(422,091)
Other income	4,241
Interest expense	(147,150)
Total other expense, net	(565,000)
Loss from operations before income taxes	(5,152,788)
Provision for income taxes	—
Net loss and comprehensive loss	$ (5,152,788)
Net loss per share attributable to common stockholders, basic and diluted	$ (0.34)
Weighted—average common shares outstanding, basic and diluted	15,270,957

The accompanying notes are an integral part of these financial statements.

Lexeo Therapeutics Inc.
Statement of changes in convertible preferred stock and stockholders' deficit

	Series A-1 preferred stock		Common units		Common stock		Additional paid-in capital	Accumulated deficit	Total stockholders' Deficit
	Outstanding shares	Amount	Outstanding shares	Amount	Outstanding shares	Amount			
Balances at January 1, 2020	—	$ —	9,500	$ 9,500	—	$ —	$ —	$ (395,037)	$ (385,537)
Issuance of common units	—	—	717	1,898	—	—	—	—	1,898
Conversion of common units to common stock	—	—	(10,217)	(11,398)	16,040,884	1,604	9,794	—	—
Issuance of Series A convertible preferred stock, net of issuance costs of $223,587	34,495,725	31,237,986	—	—	—	—	—	—	—
Net loss	—	—	—	—	—	—	—	(5,152,788)	(5,152,788)
December 31, 2020	34,495,725	$ 31,237,986	—	$ —	16,040,884	$ 1,604	$ 9,794	$ (5,547,825)	$ (5,536,427)

The accompanying notes are an integral part of these financial statements.

Lexeo Therapeutics Inc.
Statement of cash flows
For the year December 31, 2020

	2020
Cash flows from operating activities:	
Net loss	$ (5,152,788)
Adjustments to reconcile net loss to net cash (used in) provided by operating activities:	
Loss on extinguishment of Notes	422,091
Non-cash interest expense	147,150
Retirement of Cornell license obligation	1,300,000
Changes in operating assets and liabilities:	
Prepaid expenses and other assets	(34,572)
Accounts payable	44,319
Issuance costs payable	223,587
Change in deferred income	11,455
Accrued expenses and other current liabilities	(132,941)
Tax payable	38,304
Net cash (used in) provided by operating activities	(3,133,396)
Cash provided by financing activities:	
Proceeds from issuance of Series A convertible preferred stock	29,999,996
Series A convertible preferred stock issuance costs	(223,587)
Proceeds from issuance of Notes	2,602,336
Net cash provided by financing activities:	32,378,745
Net change in cash	$ 29,245,349
Cash at beginning of year	127,860
Cash at end of year	$ 29,373,209

	2020
Noncash investing and financing activities:	
Conversion of the Notes to Series A convertible preferred stock	$ 4,001,484
Preferred tranche obligation	$ 3,010,000

The accompanying notes are an integral part of these financial statements.

Lexeo Therapeutics, Inc.
Notes to financial statements

1. Description of business and basis of presentation

Description of business—LEXEO Therapeutics Inc. ("LEXEO" or the "Company") is a clinical-stage gene therapy company focused on addressing some of the most devastating genetically defined cardiovascular and central nervous system diseases affecting both larger-rare and prevalent patient populations. The Company is advancing a deep and diverse pipeline of AAV-based gene therapy candidates utilizing the Company's modular approach that integrates clinically validated technology, a disease area strategy targeting defined patient sub-populations most likely to benefit from the Company's therapies, and high-quality, scalable manufacturing, which is designed to overcome many of the challenges facing the field of gene therapy.

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—The financial statements have been prepared in accordance with accounting principles generally accepted in the U.S. ("U.S. GAAP"). Any reference in these notes to applicable guidance is meant to refer to the authoritative U.S. GAAP as found in the Accounting Standards Codification ("ASC") and Accounting Standards Update ("ASU") of the Financial Accounting Standards Board ("FASB").

Impact of COVID-19—On March 11, 2020, the World Health Organization declared the novel coronavirus ("COVID-19") outbreak a pandemic. In response to the outbreak, many jurisdictions, including those in which the Company is located, implemented measures to combat the outbreak, such as travel restrictions and shelter in place orders. The extent of the impact of the COVID-19 pandemic on the Company's business, operations and development timelines and plans remains uncertain, and will depend on certain developments, including the duration and spread of the outbreak and its impact on the Company's development activities and other third parties with whom the Company does business.

On March 27, 2020, the Coronavirus Aid, Relief, and Economic Security Act ("CARES Act") was signed into law. The CARES Act provides numerous tax provisions and other stimulus measures, including temporary suspension of certain payment requirements for the employer portion of Social Security taxes and the creation of certain refundable employee retention credits. These provisions of the CARES Act did not have a significant impact on the Company's financial statements.

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 impact of the COVID-19 pandemic, the ability to secure additional capital to fund operations and commercial success of its product candidates. Any future product candidates the Company may develop will require extensive nonclinical and clinical testing and regulatory approval prior to commercialization. These efforts require significant amounts of 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, accrual of research contract costs, and determination of fair value of the Company's convertible preferred stock and estimates of the tranche obligation liability. 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—Cash is recorded at cost, which approximates fair value. As of December 31, 2020, cash consists primarily of cash on deposit with U.S. banks denominated in U.S. dollars and investments in interest bearing money market funds. The Company regularly maintains cash balances with financial institutions which exceed Federal Deposit Insurance Corporation ("FDIC") insurance limits. At December 31, 2020, the Company had approximately $29.1 million, in excess of FDIC insured limits. The Company does not hold any cash equivalents, which would consist of highly liquid investments with original maturities of three months or less at the time of purchase.

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 may have a grant receivable balance. For the Company to be in a receivable position related to grants, the cumulative total spend on grants would have to exceed the cumulative cash receipts in the event there remains additional award money to be collected.

Debt issuance costs—Costs incurred in connection with the issuance of the Company's convertible notes have been recorded as a direct reduction against the convertible notes and amortized under the effective interest method.

Classification of convertible preferred stock—The holders of the Series A convertible preferred stock have certain liquidation rights in the event of a deemed liquidation that, in certain situations, are not solely within the control of the Company and would call for the redemption of the then outstanding Series A convertible preferred stock. Therefore, the Series A convertible preferred stock are classified outside of the stockholders' deficit on the balance sheets. The carrying value of the convertible preferred stock is not subsequently remeasured to the redemption value until the contingent redemption events are considered to be probable of occurring.

Revenue recognition—On January 1, 2020, the Company adopted ASU 2014-09, Revenue from Contracts with Customers (Topic 606), on a modified retrospective basis. Topic 606 establishes a principle for recognizing revenue upon the transfer of promised goods or services to customers, in an amount that reflects the expected consideration received in exchange for those goods or services. The standard also provides guidance on the recognition of costs related to obtaining and fulfilling customer contracts. Additionally, the standard requires disclosures regarding the nature, amount, timing and uncertainty of revenue and cash flows arising from contracts with customers. To determine revenue recognition for arrangements that the Company determines are within the scope of Topic 606, the Company performs the following five steps: (i) identify the contract(s) with a customer; (ii) identify the performance obligations in the contract; (iii) determine the transaction price; (iv) allocate the transaction price to the performance obligations in the contract; and (v) recognize revenue when (or as) the Company satisfies a performance obligation. The Company only applies the five-step model to contracts when it is probable that it will collect the consideration it is entitled to in exchange for the goods and

services it transfers to the customer. At contract inception, the Company assesses the goods or services promised within each contract that falls under the scope of Topic 606, determines those that are performance obligations and assesses whether each promised good or service is distinct. The Company then recognizes as revenue the amount of the transaction price that is allocated to the respective performance obligation when (or as) the performance obligation is satisfied. As of December 31, 2020, there is no revenue from contracts with customers.

Grant revenue—Grants received are recognized as grant revenue in the statement 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 balance sheet and grant revenue earned in excess of grant payments received is recognized as grant receivable on the balance sheets.

Research and development—Research and development costs are expensed as incurred. Research and development expenses consist of costs incurred to discover and develop drug candidates, including personnel expenses, allocated facility-related and depreciation expenses and third-party license fees in connection with nonclinical studies. Costs incurred in obtaining 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 expense consists primarily of the cost of employees to engage in corporate functions, such as finance and accounting, information technology, human resources, legal and executive management. General and administrative expense also includes rent occupancy costs, office expenses, recruiting expenses, entertainment allocations, depreciation and amortization, other general overhead costs, insurance premiums, professional service fees, and cost related to regulatory and litigation matters.

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 elected to convert 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. As of December 31, 2020, 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, 2020, the Company has not identified any uncertain tax positions.

The Company records interest and penalties related to unrecognized tax benefits in the provision for income taxes.

Segment Information—Operating segments are defined as components of an entity about which separate discrete information is available for evaluation by the chief operating decision maker, or decision-making group, in deciding how to allocate resources and in assessing performance. The Company operates and manages its business as one reportable and operating segment, which is the business of license acquisitions, regulatory approvals, clinical trials, and commercial activity related to the current portfolio of in-licensed products. The Company's chief executive officer, who is the chief operating decision maker, reviews financial information on an aggregate basis for allocating resources and evaluating financial performance.

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 described below:

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. The Company's cash balances exceed those that are federally insured. The Company's 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.

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 purpose of this calculation, 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. The Company excluded 34,495,725 potential common shares related to the conversion of Series A convertible preferred stock from the computation of diluted net loss per common share for the year ended December 31, 2020 because including them would have had an anti-dilutive effect. The Company reported a net loss for the year ended December 31, 2020.

Recently adopted accounting pronouncements—In December 2019, the FASB issued ASU 2019-12, Income Taxes (“Topic 740”): Simplifying the Accounting for Income Taxes (“ASU 2019-12”), which eliminates certain exceptions related to the approach for intra-period tax allocation, the methodology for calculating income taxes in an interim period and the recognition of deferred tax liabilities for outside basis differences. The new guidance also simplifies aspects of the accounting for franchise taxes and enacted changes in tax laws or rates and clarifies the accounting for transactions that result in a step-up in the tax basis of goodwill. The Company early adopted ASU 2019-12 effective January 1, 2019. ASU 2019-12 removes the exception to the incremental approach for intra-period tax allocation in the event of a loss from continuing operations and income or gain from other items such as other comprehensive income. The exception previously resulted in allocating a tax benefit to continuing operations and tax expense to other items, even when tax expense may have been zero. Under the simplification, no tax expense or benefit will be recorded to continuing operations. There is no impact on the Company’s financial statements for this amendment under ASU 2019-12. The other provisions within ASU 2019-12 are not applicable to the Company.

In August 2018, the FASB issued ASU 2018-13, Fair Value Measurement (“Topic 820”)—Changes to the Disclosure Requirements for Fair Value Measurement (“ASU 2018-13”), which removed the following disclosure requirements: (1) the amount of and reasons for transfers between Level 1 and Level 2 of the fair value hierarchy; (2) the policy for timing of transfers between levels; and (3) the valuation processes for Level 3 fair value measurements. Additionally, this update added the following disclosure requirements: (1) the changes in unrealized gains and losses for the period included in other comprehensive income and loss for recurring Level 3 fair value measurements held at the end of the reporting period; (2) the range and weighted average of significant unobservable inputs used to develop Level 3 fair value measurements. For certain unobservable inputs, an entity may disclose other quantitative information (such as the median or arithmetic average) in lieu of the weighted average if the entity determines that other quantitative information would be a more reasonable and rational method to reflect the distribution of unobservable inputs used to develop Level 3 fair value measurements. The Company early adopted ASU 2018-13 on January 1, 2019. For the new disclosures regarding the Company’s Level 3 fair value measurements, (see Note 3).

Recent accounting pronouncements not yet adopted— In February 2016, the FASB issued ASU 2016-02, Leases (“Topic 842”) (“ASU 2016-02”), which sets out the principles for the recognition, measurement, presentation and disclosure of leases for both parties to a contract (i.e., lessees and lessors). The new standard requires lessees to apply a dual approach, classifying leases as either finance or operating leases based on the principle of whether or not the lease is effectively a financed purchase by the lessee. This classification will determine whether lease expense is recognized based on an effective interest method or on a straight-line basis over the term of the lease. A lessee is also required to record a right-of-use asset and a lease liability for all leases with a term of greater than 12 months regardless of their classification. Leases with a term of 12 months or less may be accounted for similar to existing guidance for operating leases today. For non-public entities, ASU 2016-02 is effective for annual reporting periods beginning after December 15, 2020, including interim periods within those fiscal years, and early adoption is permitted. The Company is in the process of completing its review of its existing lease agreements under Topic 842 and does not expect the adoption of ASU 2016-02 to have a material impact on its financial position, results of operations or cash flows.

In June 2016, the FASB issued ASU 2016-13, Financial Instruments-Credit Losses (“Topic 326”): Measurement of Credit Losses on Financial Instruments (“ASU 2016-13”), which requires the measurement and recognition of

expected credit losses for financial assets held at amortized cost. ASU 2016-13 replaces the existing incurred loss impairment model with an expected loss methodology, which will result in more timely recognition of credit losses. ASU 2016-13 is effective for annual private company reporting periods, and interim periods within those years, beginning after December 15, 2023. The Company is currently in the process of evaluating the impact of the adoption of ASU 2016-13 on its financial statements.

3. Fair value measurements

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:

			Fair value measurement at December 31, 2020 using:	
	Level 1	Level 2	Level 3	Total
Assets:				
Cash	$29,373,209	$ —	$ —	$ 29,373,209
	$29,373,209	$ —	$ —	$ 29,373,209
Liabilities:				
Preferred stock tranche obligation	$ —	$ —	$ 3,010,000	$ 3,010,000
	$ —	$ —	$ 3,010,000	$ 3,010,000

The Company's preferred stock tranche obligation (see below) is carried at fair value determined according to Level 3 inputs in the fair value hierarchy as described below. The carrying values of other current assets, accounts payable and accrued expenses approximate their fair values due to the short-term nature of these assets and liabilities.

Preferred stock tranche obligation

In 2020, the Company obtained Series A funding from investors with two subsequent milestone closing dates. Each of the two milestones represent a separate tranche obligation as they may be exercised separately and an investor could transfer part of any rights under the agreement, including the right/obligation to participate in an individual tranche. The tranche obligation is recorded at fair value at the issuance date and a portion of the proceeds from the first closing equal to that amount will be allocated to the tranche obligation. At November 20, 2020, the fair value of the tranche obligation was valued at $3.0 million at a value per share equivalent to $0.05 and $0.07 for the second and third closings, respectively.

The Company determined that its obligation to issue additional shares at a fixed price (i.e. the issuance price) in subsequent tranches ("First Milestone" and "Second Milestone") (collectively, the "Milestones") following the initial closing of the Series A convertible preferred stock financings represented two freestanding financial instruments (together, the "Tranche Obligation"). The Company determined that its investors would be able to transfer their initial shares but keep the right/obligation to participate in the subsequent tranches, therefore the instruments are detachable. Further, as exercise of the tranche obligation does not result in the forfeiture of the shares issued in the initial closing, the instruments are separately exercisable. The freestanding financial instruments were classified as a liability on the Company's balance sheets and initially recorded at fair value and will be marked to market at each reporting period through the issuance of shares at the closing of the Milestones, with changes in fair value for each reporting period recognized in other income in the statement of operations (see Note 5).

In connection with the Company's initial issuance of Series A convertible preferred stock in November 2020 (see Note 5) the Company recognized the Tranche Obligation at the fair value related to each issuance, which was determined based on significant inputs not observable in the market, which represented a Level 3 measurement within the fair value hierarchy. The initial fair value of each obligation was estimated based on results of a valuation performed. The obligation will be remeasured prior to the issuance of subsequent **tranches**, and at each subsequent reporting period, as well as immediately prior to when the obligation is settled.

The Tranche Obligation was determined using the binomial pricing model, which takes into account the probability of achievement and failure of tranche milestones and issuance of subsequent shares. The Tranche Obligation is calculated based on future Series A preferred share fair value estimated as of each milestone date, and present valued using a risk-neutral probability-weighted framework at each milestone date. The value of the Series A convertible preferred stock price at each future milestone was estimated assuming that the expected stock price grows at the risk-free rate on a probability-weighted basis.

The following table summarizes information about the significant unobservable inputs used in the fair value measurements for the Tranche Obligation:

	November 20, 2020	December 31, 2020
Probability of financing	75% - 90%	75% - 90%
Probability of dissolution	10% - 25%	10% - 25%
Time to liquidity (years)	1.11 – 1.61	0.97 – 1.50
Discount rate	10% - 11%	10% - 11%

The Tranche Obligation value is discounted back to the initial issuance date and adjusted for probability of the tranche milestone achievement. In determining the fair values of the tranche obligations, estimates and assumptions impacting fair value include the estimated future values of the Company's Series A convertible preferred stock, discount rates, estimated time to liquidity, and probability of tranche closing/milestone achievement.

Conversion option liabilities

As further described in Note 4, the Company issued Notes on May 27, 2020, and August 11, 2020. The Notes contained certain features that met the definition of a derivative and were required to be bifurcated. The Company has accounted for these as a single derivative comprising all the features requiring bifurcation (the "First Conversion Option Liability" and the "Second Conversion Option Liability") (collectively the "Conversion Option Liabilities"). The fair value of the derivative liability was estimated using a scenario-based analysis comparing the probability-weighted present value of the convertible promissory note payoff at maturity with and without the bifurcated features. The Company considered possible outcomes available to the investors, including a conversion upon qualified financing transaction, and a mandatory conversion upon change of control. In addition, the probabilities applied to various scenarios, the key unobservable inputs are the time to liquidity for each scenario, and the discount rate.

The following tables summarize information about the significant unobservable inputs used in the fair value measurements for the Conversion Option Liabilities:

First Conversion Option Liability

	May 27, 2020	
	Conversion upon qualified financing transaction	Mandatory conversion upon change of control
Probability of financing	45%	45%
Probability of dissolution	0.481	3.011
Time to liquidity (years)	16.07%	15.19%
Discount rate	0.931	0.653

Second Conversion Option Liability

	August 11, 2020	
	Conversion upon qualified financing transaction	Mandatory conversion upon change of control
Probability of financing	70%	25%
Probability of dissolution	0.275	2.806
Time to liquidity (years)	12.99%	12.60%
Discount rate	0.967	0.717

In November 2020, in connection with the Company's issuance and sale of Series A convertible preferred stock, all of the outstanding principal and accrued interest under the convertible promissory notes was automatically converted into shares of Series A convertible preferred stock and the Conversion Option Liabilities were extinguished (see Note 4).

The following Level 3 table provides a roll forward of the aggregate fair values of the Company's Conversion Option Liabilities and Tranche Obligation:

Balance as of January 1, 2020	$ —
Tranche Obligation	3,010,000
First Conversion Option Liability	646,058
Second Conversion Option Liability	99,144
Extinguishment of Conversion Option Liabilities	(745,202)
Balance as of December 31, 2020	$ 3,010,000

As of December 31, 2020, there was no material remeasurement necessary.

4. Convertible notes

The Company entered into three convertible securities purchase agreements (collectively, the "Convertible Securities Agreements") on May 27, 2020, May 28, 2020, and August 11, 2020, with numerous investors to issue notes with an aggregate principal of $3.9 million (the "Notes"). The Notes included an interest rate of 6% that compounds annually, with a maturity date of the fifth anniversary of each respective note (May 27, 2025, May 28, 2025, and August 11, 2025), unless earlier converted. The Company received $2.6 million in cash and retired a $1.3 million obligation for the right to use a Cornell license (see Note 9). The Notes were issued on May 27, 2020 and August 11, 2020, and contained a conversion option feature.

The Notes are accounted for in accordance with ASC 470-20, *Debt with Conversion and Other Options* ("ASC 470-20") and ASC 815-15, *Derivatives and Hedging - Embedded Derivatives* ("ASC 815-15"). Under ASC 815-15, a feature is required to be bifurcated if all three conditions are met: (1) economic characteristics and risks of the embedded derivative are not clearly and closely related to the economic characteristics and risks of the host contract, (2) the hybrid instrument is not remeasured at fair value under otherwise applicable GAAP with changes in fair value reported in earnings as they occur, and (3) a separate instrument which the same terms as the derivative would be considered a derivative instrument subject to derivative accounting (the initial net investment for the hybrid instrument should not be considered to be the initial net investment for the derivative. The Company bifurcated the Conversion Option Liabilities as it was determined that they were required to be accounted separately as a single derivative. The initial fair value of the Conversion Option Liabilities of $0.7 million was recorded as a liability, and as a reduction to the carrying value of the Notes. On November 20, 2020, upon conversion, the Company accounted for the Conversion Option Liabilities as a gain within loss on extinguishment of notes in the statement of operations. Issuance costs related to the Notes were deemed immaterial.

The debt discount comprised of the initial fair value of the Conversion Option Liabilities is amortized using the effective interest method over the two-year contractual term of the Notes and presented as a direct reduction of the debt liability.

In November 2020, in connection with the Company's issuance and sale of Series A convertible preferred stock, all of the outstanding principal and interest under the Notes, totaling $4.0 million, with interest totaling $0.1 million recorded in interest expense in the statement of operations, was converted into 4,495,719 shares of Series A convertible preferred stock at a price ranging between 85-100% of $1.00 per share depending on the investor (see Note 5).

The Company accounted for the conversion of the Notes as a debt extinguishment and recognized a loss on extinguishment of debt of $1.2 million within other income (expense), net in the statement of operations. The loss on extinguishment was calculated as the difference between the carrying value of the Notes, net of the unamortized debt discount of $3.3 million, and the fair value of the 4,495,719 shares of Series A convertible preferred stock issued to settle the Notes of $4.5 million.

5. Preferred stock

As of December 31, 2020, the Company's certificate of incorporation authorized the Company to issue 85,500,000 shares of Series A convertible preferred stock at par value of $0.0001.

On November 20, 2020, the Company entered into a Series A convertible preferred stock purchase agreement (the "Series A Purchase Agreement") whereby the Company issued 29,999,996 shares of Series A convertible preferred stock at $1.00 per share for an aggregate purchase price of $30.0 million and incurred $0.2 million of issuance costs. Additionally, on November 20, 2020, the Company issued 4,495,719 additional shares of Series A convertible preferred stock to the holders of the Company's Notes on conversion of the Notes (see Note 4).

Pursuant to the Series A Purchase Agreement, the shareholders are eligible to purchase additional shares at the date of certain contractual milestone events. The first milestone event is the successful Federal Drug Administration ("FDA") clearance to proceed into human clinical studies of one of the Company's therapeutics. The second milestone event is the successful dosing of the therapeutic in five human patients with all relevant safety data supportive of future development. As of December 31, 2020, these milestones have not yet been achieved.

As of December 31, 2020, the Company's 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	85,500,000	34,495,725	$ 29,999,996	$ 34,495,725	34,495,725

As of December 31, 2020, the rights and privileges of the holders of the convertible preferred stock were as follows:

Voting—Holders of preferred stock are entitled 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 A convertible preferred stock are entitled to receive, when, as and if declared by the Company's board of directors, dividends at the rate of 8% per share of 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 shall be considered a "Deemed Liquidation Event" unless the holders of a majority of the outstanding shares of preferred stock elect otherwise by written notice 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 is a constituent party and the Company issues 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 continue to represent, or are converted into or exchanged for shares of capital stock that represent, immediately following such merger or consolidation, at least a majority of the capital stock of (1) the surviving or resulting company; or (2) if the surviving or resulting company is a wholly owned subsidiary of another company immediately following such merger or consolidation, the parent company of such surviving or resulting corporation; or (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 are held by such subsidiary or subsidiaries, except where such sale, lease, transfer, exclusive license or other disposition is to a wholly owned subsidiary of the Company.

Liquidation preference—In the event of any voluntary or involuntary liquidation, dissolution, or winding up of the Company, the holders of shares of preferred stock shall be 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, an amount per share equal to one times $1.00 per share, 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 shall be insufficient to pay the holders of shares of preferred stock the full amount to which they shall share 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.

Redemption—The preferred stock does not contain a mandatory redemption provision. Upon a Deemed Liquidation Event, the holders will be paid their preference amounts according to their priority. That is, the preferred stockholders will be entitled to an amount up to $1.00 per share, plus any accrued dividends. Accretion to redemption value will only be required if a Deemed Liquidation Event is probable.

Conversion—In the event that any holder of Series A preferred stock, does not participate in any Milestones then each share of preferred stock held by such holder shall automatically be converted into the number of shares of common stock equal to $1.00 divided by the product of (a) the conversion price in effect immediately prior to the consummation of the applicable Milestone closing and (b) ten.

Upon either (a) the closing of the sale of shares of common stock to the public at a price of at least $3.00 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 $50.0 million 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") or (b) the date and time, or the occurrence of an event specified by vote or written consent of the holders of a majority of the outstanding shares of Series A convertible preferred stock, then (i) all outstanding shares of preferred stock shall automatically be converted into shares of common stock at the then effective conversion rate.

6. Common units and common stock

Common units

On February 21, 2017, in connection with the formation of LEXEO Therapeutics, LLC (see Note 1), the Company issued 9,000 common units with a $1.00 par value to the founding member of the Company. Each common unit represents an interest in the Company and is entitled to distributions and allocations including profit and loss, and tax priority distributions.

In 2018, the Company issued 500 additional common units with a $1.00 par value. On January 14, 2020, and March 6, 2020, the Company issued a total of 1,898 incentive units to the Company's chief executive officer and two scientific advisors. In November 2020, the Company modified the incentive units such that the remaining service periods were eliminated, thus the incentive units were fully vested without further service conditions. As of November 20, 2020, the Company had 11,398 common units outstanding at a price of $1.00 per share. On November 20, 2020, the date of the conversion of LEXEO Therapeutics, LLC to LEXEO Therapeutics, Inc. (see Note 1), all of the common units and incentive units in the LLC were converted into common stock of the Company. Each common unitholder and incentive unitholder received a ratable ownership-percentage in the 16,040,884 shares of common stock issued at the time of the conversion.

Common stock

As of December 31, 2020, the Company's certificate of incorporation authorized the Company to issue 120,000,000 shares of common stock with a par value of $0.0001 per share. The voting, dividend and liquidation rights of the holders of the Company's common stock are subject to and qualified by the rights, powers and preferences of the holders of the convertible preferred stock set forth above.

Each share of common stock entitles the holder to one vote on all matters submitted to a vote of the Company's stockholders, provided that holders of common stock are not entitled to vote on amendments to the certificate of incorporation that relate solely to the terms of the convertible preferred stock. Common stockholders are entitled to receive dividends, if any, as may be declared by the Company's board of directors, subject to the preferential dividend rights of the convertible preferred stock. Through December 31, 2020, no cash dividends have been declared or paid by the Company.

7. 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 year ended December 31, 2020:

Federal statutory rate	21.00%
State Taxes, net of Federal benefit	6.24%
Non-taxable income	(8.24%)
Change in Valuation Allowance	(18.99%)
	0.00%

The principal components of the Company's deferred tax assets and liabilities at December 31, 2020 are as follows:

	Year ended December 31, 2020
Net operating loss carryforwards	$ 435,604
Intangible assets	543,067
Total deferred tax assets	978,670
Valuation allowance	(978,670)
Net deferred tax assets (liabilities)	$ —

As of December 31, 2020, the Company had federal net operating loss carryforwards of approximately $1.4 million, which can be carried forward indefinitely. As of December 31, 2020, the Company had state net operating loss carryforwards of approximately $1.4 million, which begin to expire in 2040.

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, 2020, 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, 2020.

Under Internal Revenue Code Section 382, if a corporation undergoes an "ownership change," the corporation's ability to use its pre-change 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 the Company became a "loss corporation" as defined in Section 382. Future changes in the Company's stock ownership, which may be outside of the Company's 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." The Company has not

conducted an “ownership change” analysis. If an “ownership change” has 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 us.

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 the Company 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 adjust these liabilities when the Company’s 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 the Company’s 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, 2020, the Company has not recorded any uncertain tax positions in the Company’s financial statements.

The Company recognizes interest and penalties related to unrecognized tax benefits on the income tax expense line in the accompanying statement of operations. As of December 31, 2020, no accrued interest or penalties are included on the related tax liability line in the 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 financial statements.

8. Commitments and contingencies

Leases—In January 2017, the Company entered into a lease agreement for office space at 364 E 69th Street, New York, NY, which expired January 30, 2020. The monthly lease payments were $1,500.

In January 2020, the Company entered into a lease agreement for office space with LaunchLabs at 430 E 29th St, New York, NY. As per the terms of the lease, the Company is eligible to renew annually. Monthly lease payments of $1,295 are inclusive of license and membership fees.

Contingencies—From time to time, the Company may have certain contingent liabilities that arise in the ordinary course of business. The Company accrues a liability for such 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.

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 will enter into indemnification agreements with its 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. As of December 31, 2020, the Company had not incurred any material costs as a result of such indemnifications.

9. License agreements

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, Alzheimer's disease, and Anti-Tau, although our license is not restricted by such indications and it includes assignment to us of Cornell's IND for the use of AAVrh10.hAPOE vector to treat *APOE4* homozygous patients who are at risk for or have Alzheimer's disease. The Cornell Second License Agreement is for the in-license of technology related to portfolios for infantile neuronal ceroid lipofuscinosis (also called CLN2 Batten disease) and Friedreich's ataxia although our license is not restricted by such indications, and it includes assignment to us of Cornell's IND for the use of AAVrh.10CUhCLN2 to treat children with CLN2 Batten disease. Through the Cornell License Agreements, the Company gains access to a portfolio of inventions, patent rights, technology, and licensed methods that the Company plans 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 (see Note 4). In November 2020, Notes with outstanding principal of $1.3 million were cancelled in exchange for 1,337,610 shares of Series A convertible 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 upon the achievement of specific clinical and regulatory milestones under the Cornell Second License Agreement. The Company achieved the first clinical milestone under the Cornell Second License Agreement when it submitted its IND for LX2006 to the FDA. A payment of $0.1 million is due to Cornell in connection with this milestone. 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 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.

10. Related parties

On May 27, 2020, the Company entered into two executive license agreements with and subsequently issued $1.3 million in Notes to Cornell (see Note 9 and Note 4). As of December 31, 2020, Cornell owns more than 2.6% of the Company's outstanding Series A convertible preferred stock. These transactions are related party transactions.

11. Subsequent events

The Company has reviewed and evaluated subsequent events through September 8, 2021, the date that the financial statements were available to be issued.

Adverum Biotechnologies Inc. Exclusive License Agreement—On January 25, 2021, the Company entered into an exclusive license agreement with Adverum Biotechnologies Inc. ("Adverum") (the "Adverum Agreement") to in-license materials and technology related to the treatment of cardiomyopathy due to Friedreich's ataxia. In connection with the Adverum Agreement, the Company will gain access to a portfolio of inventions, patent rights, technology, and licensed methods that the Company plans to develop, and the Company

assumed all worldwide development and commercialization activities. The Company is obligated to pay Adverum up to $17.5 million upon the achievement of specified development and regulatory milestones and up to $49.0 million in commercialization and sales milestones for the products. The Company is obligated to pay Adverum tiered royalties ranging from a rate in the high single-digits to 10% 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 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.

Weill Cornell Medical College Research Collaboration Agreement—On February 2, 2021, the Company entered into a Research Collaboration Agreement with Weill Cornell Medical College ("WCM") (the "WCM Agreement") in conjunction with the Cornell License Agreements entered on May 27, 2020. The Company committed to fund scientific research at WCM related to the intellectual property licensed to the Company pursuant to the Cornell License Agreements.

Under the terms of the WCM Agreement, the Company will pay WCM a total of $3.0 million over the course of three years, with equal $0.25 million payments due each quarter starting 10 days after the execution of the WCM Agreement. With respect to each WCM invention, joint invention, and related joint results for which an Improvement applies and the Company has made an election to amend the 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.

The WCM Agreement terminates upon the expiration of the agreed upon three-year term. In addition, the WCM Agreement may be terminated by the Company in the event that the WCM principal investigator ceases to supervise the research and WCM is unable to or declines to find a substitute.

Stock based compensation plan—In February 2021, the Company adopted the 2021 Equity Incentive Plan (the "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. The purposes of the Plan 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 Plan authorizes up to 8,918,225 of shares of the Company's common stock to be issued.

Stelios Therapeutics, Inc. Acquisition—On July 16, 2021, the Company acquired all issued and outstanding stock of Stelios Therapeutics, Inc., for initial consideration of $7.0 million, with up to an aggregate of $20.5 million payable upon the achievement of certain development milestones. The Company accounted for the acquisition of Stelios as an asset acquisition pursuant to Financial Accounting Standards Board Accounting Standards Codification Section 805, *Business Combinations*. The Company acquired in-process research and development, or IPR&D, assets from Stelios related to arrhythmogenic right ventricular cardiomyopathy and TNNI3-associated hypertrophic cardiomyopathy programs. 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.

Series A convertible preferred stock—On July 30, 2021, the Company's board of directors and stockholders voted to waive the milestones related to both the second and third tranches of the Series A convertible preferred stock and issued 50,999,997 shares for an aggregate consideration of $51.0 million.

Series B convertible preferred stock—On August 10, 2021, the Company entered into a Series B convertible preferred stock purchase agreement whereby the Company issued 58,128,763 shares of Series B convertible preferred stock at $1.72 per share for an aggregate purchase price of approximately $100.1 million.

Shares

Common Stock

J.P. Morgan **SVB Leerink** **Stifel** **RBC Capital Markets**

Chardan

Through and including , 2022 (the 25th day after the date of this prospectus), all dealers that buy, sell or trade shares of our common stock, whether or not participating in this offering, may be required to deliver a prospectus. This delivery requirement is in addition to the dealers' obligation to deliver a prospectus when acting as underwriters and with respect to their unsold allotments or subscriptions.

Part II

Information Not Required in Prospectus

Item 13. Other Expenses of Issuance and Distribution.

The following table indicates the expenses to be incurred in connection with the offering described in this registration statement, other than underwriting discounts and commissions, all of which will be paid by us. All amounts are estimated except the Securities and Exchange Commission, or SEC, registration fee, the Financial Industry Regulatory Authority, Inc., or FINRA, filing fee and the Nasdaq Global Market initial listing fee.

	Amount
SEC registration fee	$ *
FINRA filing fee	*
Nasdaq Global Market initial listing fee	*
Accountants' fees and expenses	*
Legal fees and expenses	*
Blue sky fees and expenses	*
Transfer agent's fees and expenses	*
Printing and engraving expenses	*
Miscellaneous	*
Total expenses	$ *

* To be provided by amendment

Item 14. Indemnification of Directors and Officers.

We are incorporated under the laws of the State of Delaware. Section 102 of the Delaware General Corporation Law permits a corporation to eliminate the personal liability of directors of a corporation to the corporation or its stockholders for monetary damages for a breach of fiduciary duty as a director, except where the director breached his duty of loyalty, failed to act in good faith, engaged in intentional misconduct or knowingly violated a law, authorized the payment of a dividend or approved a stock repurchase in violation of Delaware corporate law or obtained an improper personal benefit.

Section 145 of the Delaware General Corporation Law provides that a corporation has the power to indemnify a director, officer, employee or agent of the corporation and certain other persons serving at the request of the corporation in related capacities against expenses (including attorneys' fees), judgments, fines and amounts paid in settlements actually and reasonably incurred by the person in connection with an action, suit or proceeding to which he is or is threatened to be made a party by reason of such position, if such person acted in good faith and in a manner he reasonably believed to be in or not opposed to the best interests of the corporation, and, in any criminal action or proceeding, had no reasonable cause to believe his conduct was unlawful, except that, in the case of actions brought by or in the right of the corporation, no indemnification shall be made with respect to any claim, issue or matter as to which such person shall have been adjudged to be liable to the corporation unless and only to the extent that the Court of Chancery or other adjudicating court determines that, despite the adjudication of liability but in view of all of the circumstances of the case, such person is fairly and reasonably entitled to indemnity for such expenses which the Court of Chancery or such other court shall deem proper.

As permitted by the Delaware General Corporation Law, our amended and restated certificate of incorporation and bylaws to be in effect upon the closing of this offering will provide that: (i) we are required to indemnify our

directors to the fullest extent permitted by the Delaware General Corporation Law; (ii) we may, in our discretion, indemnify our officers, employees and agents as set forth in the Delaware General Corporation Law; (iii) we are required, upon satisfaction of certain conditions, to advance all expenses incurred by our directors in connection with certain legal proceedings; (iv) the rights conferred in the bylaws are not exclusive; and (v) we are authorized to enter into indemnification agreements with our directors, officers, employees and agents.

In connection with this offering, we expect to enter into indemnification agreements with each of our directors and executive officers that require us to indemnify them against expenses, judgments, fines, settlements and other amounts that any such person becomes legally obligated to pay (including with respect to a derivative action) in connection with any proceeding, whether actual or threatened, to which such person may be made a party by reason of the fact that such person is or was a director or officer of us or any of our affiliates, provided such person acted in good faith and in a manner such person reasonably believed to be in, or not opposed to, our best interests. The indemnification agreements will also set forth certain procedures that will apply in the event of a claim for indemnification thereunder. We intend to enter into similar indemnification agreements with our executive officers prior to the completion of this offering. At present, no litigation or proceeding is pending that involves any of our directors or officers regarding which indemnification is sought, nor are we aware of any threatened litigation that may result in claims for indemnification.

We maintain a directors' and officers' liability insurance policy. The policy insures directors and officers against unindemnified losses arising from certain wrongful acts in their capacities as directors and officers and reimburses us for those losses for which we have lawfully indemnified the directors and officers. The policy contains various exclusions.

In addition, the underwriting agreement filed as Exhibit 1.1 to this Registration Statement provides for indemnification by the underwriters of us and our officers and directors for certain liabilities arising under the Securities Act, or otherwise. Our amended and restated investor rights agreement with certain investors also provides for cross-indemnification in connection with the registration of our common stock on behalf of such investors.

Item 15. Recent Sales of Unregistered Securities.

The following list sets forth information regarding all unregistered securities sold by us since our inception through the date of the prospectus that forms a part of this registration statement.

Issuances of Limited Liability Interests

In connection with our formation in February 2017, we issued 95 limited liability common units to our founder and 5 limited liability common units to a service provider that is an accredited investor.

In January 2020, we issued 500 limited liability company incentive units to our chief executive officer.

In May 2020, we issued an aggregate of 206 limited liability company incentive units to two of our employees.

Issuances of Common Stock

In November 2020, we issued an aggregate of 16,040,884 shares of our common stock to five individuals and accredited investors, including our founder and our chief executive officer, in connection with the conversion of all outstanding units to shares of common stock and the conversion of our company from a limited liability company to a corporation.

Issuances of Convertible Promissory Notes

In May 2020, we issued a convertible promissory note to Cornell University in the principal amount of $650,000.

In May 2020, we issued a convertible promissory note to Cornell University in the principal amount of $650,000.

In June 2020, we issued a convertible promissory note to the Alzheimer's Drug Discovery Foundation in the principal amount of $1,977,336.

In August 2020, we issued a convertible promissory note to the Alchemy LP in the principal amount of $250,000.

In August 2020, we issued a convertible promissory note to Thomas McWilliams in the principal amount of $250,000.

In September 2020, we issued a convertible promissory note to Nova Venture Holdings LLC in the principal amount of $125,000.

Issuances of Preferred Stock

In November 2020, we issued an aggregate of 31,337,606 shares of our Series A convertible preferred stock to 13 investors at a purchase price of $1.00 per share, for aggregate cash consideration of approximately $30 million and pursuant to the conversion of certain convertible promissory notes and related cancellation of indebtedness of approximately $1.3 million. We also issued an aggregate of 4,495,729 shares of our Series A convertible preferred stock to four investors at a price of $0.85 per share, pursuant to the conversion of certain convertible promissory notes and related cancellation of indebtedness of approximately $2.7 million.

In July 2021, we issued an aggregate of 50,999,997 shares of our Series A convertible preferred stock to 11 investors at a purchase price of $1.00 per share, for aggregate consideration of approximately $51 million.

In August 2021, we issued an aggregate of 58,157,823 shares of our Series B convertible preferred stock to 32 investors at a purchase price of $1.72049 per share, for aggregate consideration of over $100 million.

Issuances Pursuant to our Equity Plans

From February 2017 (the date of our inception) through the date of this registration statement, we granted options under the Existing Plan to purchase an aggregate of shares of common stock, at an exercise price of $ per share, to our employees and consultants. Of these, shares have been issued upon the exercise of options, and options have been forfeited, expired or cancelled.

None of the foregoing transactions involved any underwriters, underwriting discounts or commissions, or any public offering. Unless otherwise specified above, we believe these transactions were exempt from registration under the Securities Act in reliance on Sections 3(a)(9) and 4(a)(2) of the Securities Act (and Regulation D or Regulation S promulgated thereunder) or Rule 701 promulgated under Section 3(b) of the Securities Act as transactions by an issuer not involving any public offering or under benefit plans and contracts relating to compensation as provided under Rule 701. The recipients of the securities in each of these transactions represented their intentions to acquire the securities for investment only and not with a view to or for sale in connection with any distribution thereof, and appropriate legends were placed on the share certificates issued in these transactions. All recipients had adequate access, through their relationships with us, to information about us. The sales of these securities were made without any general solicitation or advertising.

Item 16. Exhibits and Financial Statement Schedules.

(a) Exhibits.

The exhibits listed below are filed as part of this registration statement.

Exhibit Number	Description of Exhibit
1.1*	Form of Underwriting Agreement
3.1*	Amended and Restated Certificate of Incorporation of the Registrant (as amended and currently in effect)
3.2*	Bylaws of the Registrant (currently in effect)
3.3*	Form of Amended and Restated Certificate of Incorporation of the Registrant (to be effective upon the closing of this offering)
3.4*	Form of Amended and Restated Bylaws of the Registrant (to be effective upon the closing of this offering)
4.1*	Amended and Restated Investors' Rights Agreement, by and among the Registrant and certain of its stockholders, dated August 10, 2021
5.1*	Opinion of Cooley LLP
10.1+*	2021 Equity Incentive Plan and Forms of Option Grant Notice and Agreement, Exercise Notice, Early Exercise Notice and Restricted Stock Award Notice
10.2+*	2022 Stock Incentive Plan and Forms of Stock Option Grant Notice, Stock Option Agreement, Restricted Stock Unit Grant Notice and Restricted Stock Unit Award Agreement
10.3+*	2022 Employee Stock Purchase Plan
10.4+*	Form of Indemnification Agreement with Executive Officers and Directors
10.5†*	First License Agreement, dated May 28, 2020, between LEXEO Therapeutics, LLC and Cornell University
10.6†*	Second License Agreement, dated May 28, 2020, between LEXEO Therapeutics, LLC and Cornell University
10.7†*	License Agreement, dated January 19, 2021, between Adverum Biotechnologies, Inc. and LEXEO Therapeutics, LLC
23.1*	Consent of KPMG LLP, independent registered public accounting firm
23.2*	Consent of Cooley LLP (included in Exhibit 5.1)
24.1*	Power of Attorney (included on signature page)
107*	Filing Fee Table

+ Indicates management contract or compensatory plan.

† Confidential treatment will be requested for portions of this agreement.

* To be filed by amendment.

(b) Financial Statement Schedules.

Schedules not listed above have been omitted because the information required to be set forth therein is not applicable or is shown in the financial statements or notes thereto.

Item 17. Undertakings.

The undersigned Registrant hereby undertakes to provide to the underwriters at the closing specified in the underwriting agreement, certificates in such denominations and registered in such names as required by the underwriters to permit prompt delivery to each purchaser.

Insofar as indemnification for liabilities arising under the Securities Act may be permitted to directors, officers and controlling persons of the Registrant pursuant to the foregoing provisions, or otherwise, the Registrant has been advised that in the opinion of the SEC such indemnification is against public policy as expressed in the Securities Act and is, therefore, unenforceable. In the event that a claim for indemnification against such liabilities (other than the payment by the Registrant of expenses incurred or paid by a director, officer or controlling person of the Registrant in the successful defense of any action, suit or proceeding) is asserted by such director, officer or controlling person in connection with the securities being registered, the Registrant will, unless in the opinion of its counsel the matter has been settled by controlling precedent, submit to a court of appropriate jurisdiction the question whether such indemnification by it is against public policy as expressed in the Securities Act and will be governed by the final adjudication of such issue.

The undersigned Registrant hereby undertakes that:

(1) For purposes of determining any liability under the Securities Act, the information omitted from the form of prospectus filed as part of this Registration Statement in reliance upon Rule 430A and contained in a form of prospectus filed by the Registrant pursuant to Rule 424(b)(1) or (4) or 497(h) under the Securities Act shall be deemed to be part of this Registration Statement as of the time it was declared effective.

(2) For the purpose of determining any liability under the Securities Act, each post-effective amendment that contains a form of prospectus shall be deemed to be a new registration statement relating to the securities offered therein, and the offering of such securities at that time shall be deemed to be the initial bona fide offering thereof.

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Signatures

Pursuant to the requirements of the Securities Act of 1933, the registrant has duly caused this registration statement to be signed on its behalf by the undersigned, thereunto duly authorized in the City of New York, State of New York, on this day of , 2022.

LEXEO THERAPEUTICS, INC.

By: _____
R. Nolan Townsend
Chief Executive Officer

KNOW ALL BY THESE PRESENTS, that each person whose signature appears below hereby constitutes and appoints R. Nolan Townsend as his or her true and lawful agent, proxy and attorney-in-fact, with full power of substitution and resubstitution, for him or her and in his or her name, place and stead, in any and all capacities, to (i) act on, sign and file with the Securities and Exchange Commission any and all amendments (including post-effective amendments) to this registration statement together with all schedules and exhibits thereto and any subsequent registration statement filed pursuant to Rule 462(b) under the Securities Act of 1933, as amended, together with all schedules and exhibits thereto, (ii) act on, sign and file such certificates, instruments, agreements and other documents as may be necessary or appropriate in connection therewith, (iii) act on and file any supplement to any prospectus included in this registration statement or any such amendment or any subsequent registration statement filed pursuant to Rule 462(b) under the Securities Act of 1933, as amended, and (iv) take any and all actions which may be necessary or appropriate to be done, as fully for all intents and purposes as he might or could do in person, hereby approving, ratifying and confirming all that such agent, proxy and attorney-in-fact or any of his substitutes may lawfully do or cause to be done by virtue thereof.

Pursuant to the requirements of the Securities Act of 1933, this registration statement has been signed by the following persons in the capacities and on the dates indicated.

Signature	Title	Date
_____ R. Nolan Townsend	Chief Executive Officer and Director *(Principal Executive Officer)*	, 2022
_____ R. Nolan Townsend	*(Principal Financial and Accounting Officer)*	, 2022
_____ Mette Kirstine Agger	Director	, 2022
_____ Steven Altschuler, M.D.	Director	, 2022
_____ Paula HJ Cholmondeley	Director	, 2022
_____ Bernard Davitian	Director	, 2022
_____ Reinaldo Diaz	Director	, 2022