Lexeo Therapeutics Announces License Agreement to Accelerate Development of LX2006 for the Treatment of Friedreich Ataxia Cardiomyopathy

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Lexeo Therapeutics gains intellectual property rights including current and future clinical data from ongoing Weill Cornell Medicine investigator-initiated trial of gene therapy candidate AAVrh.10hFXN (LX2006) to support regulatory discussions

Together with Lexeo Therapeutics' ongoing study of LX2006, a total of 11 participants have been treated to date; includes patients at treatment durations out to 18-months with no treatment-related serious adverse events observed across either study

Interim readout of combined data set at multiple doses expected mid-year 2024

NEW YORK, April 22, 2024 (GLOBE NEWSWIRE) -- Lexeo Therapeutics, Inc. (Nasdaq: LXEO), a clinical stage genetic medicine company, today announced an in-license agreement with Cornell University to expedite development of the investigational gene therapy candidate LX2006 for the treatment of Friedreich ataxia (FA) cardiomyopathy.

Under the license agreement, Lexeo has acquired certain rights[1] including rights to current and future data generated in an ongoing investigator-initiated Phase 1A trial of AAVrh.10hFXN to treat FA cardiomyopathy (NCT05302271). The agreement will support Lexeo's efforts to develop a potentially life-changing therapy for this unmet need.

The investigator-initiated trial is being conducted by Weill Cornell Medicine, which has pioneered groundbreaking research on the potential of gene therapy in FA, published preclinical data that supported the first ever gene therapy IND clearance for FA, and sponsored a natural history study for almost a decade to better characterize the condition and its progression. Lexeo previously licensed know-how relating to AAVrh.10hFXN from Weill Cornell Medicine and collaborated with researchers there to further study the candidate, which Lexeo refers to as LX2006. Lexeo is studying LX2006 in the company-sponsored, open label, dose-ascending, multicenter SUNRISE-FA Phase 1/2 trial (NCT05445323), in which four patients have been dosed to date across cohorts 1 & 2. Weill Cornell Medicine has dosed seven patients to date with LX2006 across dose cohorts 1 & 2 and is collecting biomarker, structural, and functional cardiac data akin to SUNRISE-FA.

"The larger aggregate data set, combined with Orphan Drug, Rare Pediatric Disease, and Fast Track designations from FDA, is anticipated to facilitate an accelerated path to regulatory engagements for LX2006," said R. Nolan Townsend, Chief Executive Officer of Lexeo Therapeutics. "We are excited about the opportunity to advance research in FA cardiomyopathy, which is the leading cause of death in FA and has no approved treatment options today."

The interim clinical data readout of LX2006 is expected mid-year 2024. With the newly-licensed data, the readout will now include participants across the two studies, approximately doubling the number of evaluable patients and including patients with a treatment duration out to 18-months. Lexeo also expects to provide an analysis of natural history data and baseline characteristics for study participants from both studies to characterize the cardiovascular disease phenotype seen in FA cardiomyopathy ahead of the interim readout.

"This agreement with Lexeo Therapeutics builds upon years of collaboration between Weill Cornell Medicine and Lexeo to benefit patients with FA cardiomyopathy. It is our intention that this license agreement will accelerate the clinical investigation and development of LX2006 as a potential life-saving therapy for patients with FA," said Dr. Lisa Placanica, Senior Managing Director, Center for Technology Licensing at Weill Cornell Medicine.

Patients Treated with LX2006 Across Clinical Trials, as of April 22, 2024

Dose	Combined Enrollment Update and Months of Follow-Up		
	>12 months	**6-12 months**	**<6 months**
Dose Cohort 1 $1.8x10^{11}$ vg/kg	3	3	-
Dose Cohort 2 $5.6x10^{11}$ vg/kg	-	2	3

Note: Cardiac biopsies are performed only in the SUNRISE-FA trial; one patient in dose cohort 1 and three patients in dose cohort 2 have undergone cardiac biopsies.

The Phase 1A study of AAVrh.10hFXN conducted by investigators at Weill Cornell Medicine is a single-site, 52-week, dose-ascending, open-label trial evaluating the safety and preliminary efficacy of AAVrh.10hFXN in patients who have FA cardiomyopathy. AAVrh.10hFXN is administered as a one-time intravenous infusion to patients in two ascending-dose cohorts with five participants per cohort. While cardiac biopsies are not collected in this study, key cardiac disease measures are collected at 3, 6 and 12-month intervals and complement data collected in SUNRISE-FA.

SUNRISE-FA is a multicenter, 52-week, dose-ascending, open-label trial evaluating the safety and preliminary efficacy of LX2006 in patients who have FA cardiomyopathy. LX2006 is administered as a one-time intravenous infusion to patients in at least two ascending-dose cohorts with the potential to escalate to a third cohort at a dose of $1.2x10^{12}$ vg/kg. Long-term safety and efficacy will be evaluated for five years following dosing in both trials.

[1]The license agreement includes a package of intellectual property rights including know-how previously licensed to the Company, patent rights related to LX2006, and rights to current and future data generated in an ongoing investigator-initiated Phase 1A trial of AAVrh.10hFXN to treat FA cardiomyopathy (NCT05302271).

About LX2006
LX2006 is an AAV-based gene therapy candidate delivered intravenously for the treatment of FA cardiomyopathy, the most common cause of mortality in patients with FA affecting approximately 5,000 patients in the United States. LX2006 is designed to target the cardiac manifestations of FA by delivering a functional frataxin gene to promote the expression of the frataxin protein and restore mitochondrial function in myocardial cells. In preclinical studies, LX2006 reversed the cardiac abnormalities in FA disease models and showed improvement in cardiac function and survival while demonstrating a favorable safety profile. The FDA has granted Rare Pediatric Disease designation, Fast Track designation, and Orphan Drug designation to LX2006 for the treatment of FA cardiomyopathy.

About Lexeo Therapeutics
Lexeo Therapeutics is a New York City-based, clinical stage genetic medicine company dedicated to transforming healthcare by applying pioneering science to fundamentally change how genetically defined cardiovascular diseases and APOE4-associated Alzheimer's disease are treated. Using a stepwise development approach, Lexeo is leveraging early proof-of-concept functional and biomarker data to advance a pipeline of cardiovascular and APOE4-associated Alzheimer's disease programs.

Cautionary Note Regarding Forward-Looking Statements
Certain statements in this press release may constitute "forward-looking statements" within the meaning of the federal securities laws, including, but not limited to, our expectations and plans regarding our current product candidates and programs, including statements regarding the anticipated benefits of the license agreement between Lexeo Therapeutics and Cornell University and the data to be provided thereunder, including the acceleration of the development of our product candidates and the timing of approvals, if any. Words such as "may," "might," "will," "objective," "intend," "should," "could," "can," "would," "expect," "believe," "design," "estimate," "predict," "potential," "develop," "plan" or the negative of these terms, and similar expressions, or statements regarding intent, belief, or current expectations, are forward-looking statements. While Lexeo believes these forward-looking statements are reasonable, undue reliance should not be placed on any such forward-looking statements. These forward-looking statements are based upon current information available to the company as well as certain estimates and assumptions and are subject to various risks and uncertainties (including, without limitation, those set forth in Lexeo's filings with the U.S. Securities and Exchange Commission (SEC)), many of which are beyond the company's control and subject to change. Actual results could be materially different from those indicated by such forward looking statements as a result of many factors, including but not limited to: risks and uncertainties related to global macroeconomic conditions and related volatility; expectations regarding the initiation, progress, and expected results of Lexeo's preclinical studies, clinical trials and research and development programs; the unpredictable relationship between preclinical study results and clinical study results; delays in submission of regulatory filings or failure to receive regulatory approval; liquidity and capital resources; and other risks and uncertainties identified in Lexeo's Annual Report on Form 10-K for the annual period ended December 31, 2023, filed with the SEC on March 11, 2024, and subsequent future filings Lexeo may make with the SEC. New risks and uncertainties may emerge from time to time, and it is not possible to predict all risks and uncertainties. Lexeo claims the protection of the Safe Harbor contained in the Private Securities Litigation Reform Act of 1995 for forward-looking statements. Lexeo expressly disclaims any obligation to update or alter any statements whether as a result of new information, future events or otherwise, except as required by law.

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