Lexeo Therapeutics Granted FDA Fast Track Designation for LX2006, an AAV-Based Gene Therapy Candidate for the Treatment of Friedreich's Ataxia Cardiomyopathy

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NEW YORK, April 16, 2024 (GLOBE NEWSWIRE) -- Lexeo Therapeutics, Inc. (Nasdaq: LXEO), a clinical stage genetic medicine company dedicated to pioneering treatments for genetically defined cardiovascular diseases and APOE4-associated Alzheimer's disease, today announced the U.S. Food and Drug Administration (FDA) has granted Fast Track designation to LX2006, the company's AAVrh.10hFXN-based gene therapy candidate for the treatment of Friedreich's ataxia (FA) cardiomyopathy. LX2006 is designed to deliver a functional frataxin gene to promote frataxin protein expression and restore mitochondrial function in myocardial cells.

Fast Track is a process designed to facilitate the development and expedite the review of new drugs intended to treat serious conditions and address unmet medical need. This designation was granted based on available preclinical data. SUNRISE-FA, a Phase 1/2 multicenter, 52-week, dose-ascending, open-label clinical trial, is ongoing to evaluate the safety and tolerability, as well as preliminary efficacy, of LX2006 in patients with FA cardiomyopathy.

"FA cardiomyopathy is the leading cause of death among FA patients, and there are currently no approved treatment options. The FDA's Fast Track designation for LX2006 underscores the significant unmet need for effective treatment options to address the cardiac impact of this debilitating disease," said R. Nolan Townsend, Chief Executive Officer of Lexeo Therapeutics. "We believe today's Fast Track designation, along with the previously announced Rare Pediatric Disease and Orphan Drug designations granted to LX2006, will allow for enhanced regulatory interactions and the potential for this life-improving therapy to reach FA patients more quickly."

LX2006 is administered as a one-time intravenous infusion to patients in at least two ascending-dose cohorts with the potential for a third cohort. Long-term safety and efficacy will be evaluated for an additional four years following completion of the initial year of the trial, resulting in data from a total of five years post-LX2006 treatment.

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 Fast Track designation, Rare Pediatric Disease 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 timing of the initiation of and results from our clinical trials and other information that is not historical information. 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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