Lexeo Therapeutics Announces Publication in JAMA Cardiology of Phase I/II Data for LX2006 in Friedreich Ataxia

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Published findings in JAMA Cardiology demonstrate LX2006 generally well tolerated, with early signs of efficacy in Phase I/II studies

SUNRISE-FA 2 pivotal study for LX2006 on track to initiate in Q2 2026 with topline data expected in 2H 2027

NEW YORK, June 17, 2026 (GLOBE NEWSWIRE) -- Lexeo Therapeutics, Inc. (Nasdaq: LXEO), a clinical stage genetic medicine company dedicated to pioneering novel treatments for cardiovascular diseases, today announced that key results from Phase I/II studies of LX2006 gene therapy in Friedreich ataxia (FA) have been published in the Journal of the American Medical Association (JAMA) Cardiology, linked here.

JAMA Cardiology Publication

The assessment of safety and exploratory efficacy parameters of LX2006 combines data from two independent studies: nine participants from a Weill Cornell Medicine study, funded by the National Heart, Lung, and Blood Institute, and eight participants treated in the SUNRISE-FA study carried out by Lexeo Therapeutics. The two studies included patients with early cardiac disease and patients with established structural cardiac disease. In both studies, the patients received a one-hour intravenous infusion of LX2006 gene therapy and were evaluated from 6 to 36 months. Three different doses were tested among three cohorts of patients.

“These positive Phase I/II data demonstrate clinically meaningful improvements across both cardiac and neurologic measures of Friedreich ataxia and this publication in JAMA Cardiology further underscores the significance of these results and the potential of this therapy for individuals living with this devastating disease,” said Narinder Bhalla, M.D., Chief Medical Officer of Lexeo Therapeutics. “We are grateful to Weill Cornell Medicine and the study investigators for helping advance this program to the next stage of clinical development and are excited to initiate the pivotal SUNRISE-FA 2 study this month.”

“The publication of these results in JAMA Cardiology represents a meaningful milestone for our research program. Friedreich ataxia cardiomyopathy remains a progressive and life-threatening condition with no approved cardiac-specific treatments, and these findings reinforce the potential of gene therapy to address the underlying cause of disease,” said Dr. Ronald G. Crystal, lead author, professor and chair of the Department of Genetic Medicine at Weill Cornell Medicine and a pulmonologist at New-York-Presbyterian/Weill Cornell Medical Center. “I look forward to building on these results as the program advances towards a pivotal study and ultimately, an approved therapy for patients.”

Key Findings from LX2006 Phase I/II Studies

LX2006 clinical data to date show sustained or deepening improvements across cardiac and neurologic outcomes of FA as well as improvement in frataxin biomarker expression.

- The majority of participants demonstrated LVMI improvement or stabilization over time. Among participants with abnormal baseline LVMI in mid- and high-dose cohorts (n=3), there was a 28% mean improvement in LVMI at 6 months and 33% mean improvement at 12 months. Some patients maintained LVMI improvement out to three years following treatment, demonstrating sustained disease modification across a clinically meaningful endpoint.
- The improvement or stabilization in secondary cardiac biomarkers, high-sensitivity troponin I and lateral wall thickness, was observed in most patients independent of baseline LVMI, supporting LX2006’s potential across stages of FA-cardiomyopathy.
- Cardiac biopsy data from the SUNRISE-FA trial (n=8) showed that all study participants achieved increases in frataxin protein expression from baseline at 3 months, marking the first evidence of meaningful expression in disease-relevant cardiac tissue.
- LX2006 was also associated with stabilization over time of the modified Friedreich Ataxia Rating Scale (mFARS), suggesting evidence of neurological functional improvement.
- Treatment with LX2006 was generally well-tolerated across 17 participants dosed, with no Grade 3+ serious adverse events (SAEs) to date, no clinically significant complement activation, and minimal, transient liver function test (LFT) elevations. One patient experienced a possibly treatment-related Grade 2 event of asymptomatic myocarditis observed one year after dosing.

LX2006 will continue to be evaluated in the SUNRISE-FA 2 pivotal study, which is on track to initiate by the end of June. Materials related to the recently announced registrational trial design for LX2006 are available in the Investors section of Lexeo’s website.

Dr. Ronald G. Crystal is a founder, chief scientific advisor, consultant, equity holder and board observer of Lexeo Therapeutics and is an inventor on intellectual property assigned to Weill Cornell Medicine. Weill Cornell Medicine Enterprise Innovation, which aims to accelerate the translation of scientific discoveries into patient impact, played a crucial role in launching Lexeo in 2020 and later licensed to it additional technology to further support the clinical trial.

About Lexeo Therapeutics
Lexeo Therapeutics is a New York City-based, clinical stage genetic medicine company dedicated to reshaping heart health by applying pioneering science to fundamentally change how cardiovascular diseases are treated. The Company is advancing a portfolio of therapeutic candidates that take

aim at the underlying genetic causes of conditions, including LX2006 in Friedreich ataxia (FA), LX2020 in plakophilin-2 (PKP2) arrhythmogenic cardiomyopathy, and others in devastating diseases with high unmet need.

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, Lexeo's expectations and plans regarding its current product candidates and programs, the anticipated benefits of its current product candidates, and the timing and likelihood of potential regulatory developments and approvals. 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: the outcome of ongoing discussions with the FDA regarding the design of our pivotal trial and full approval study; 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 Quarterly Report on Form 10-Q for the quarterly period ended March 31, 2026, filed with the SEC on May 11, 2026, 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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