Lexeo Therapeutics Announces FDA Breakthrough Therapy Designation for LX2006 in Friedreich Ataxia

July 7, 2025

Breakthrough Therapy designation based on interim clinical data from Phase I/II trials showing clinically meaningful improvements in cardiac biomarkers and functional measures

LX2006 also selected for FDA Chemistry, Manufacturing, and Controls Development and Readiness Pilot (CDRP) program, created to facilitate CMC registrational readiness and support faster patient access

NEW YORK, July 07, 2025 (GLOBE NEWSWIRE) -- Lexeo Therapeutics, Inc. (Nasdaq: LXEO), a clinical stage genetic medicine company dedicated to pioneering novel treatments for cardiovascular diseases, today announced that the U.S. Food and Drug administration (FDA) has granted Breakthrough Therapy designation to LX2006 based on clinical evidence generated on both cardiac and neurologic measures of Friedreich ataxia (FA). LX2006 has also been selected to participate in the FDA Chemistry, Manufacturing, and Controls (CMC) Development and Readiness Pilot (CDRP) program, intended to enable earlier patient access to therapies with expedited clinical development timelines.

“Receiving Breakthrough Therapy designation is a significant milestone, highlighting the potential of LX2006 and the strength of clinical evidence generated to date,” said Dr. Sandi See Tai, Chief Development Officer of Lexeo Therapeutics. “We are highly encouraged by the impact of LX2006 on key measures of cardiac health, especially given the lack of treatments for FA cardiomyopathy today, which is the leading cause of death in FA. We are also optimistic about the improvements we have observed in functional measures of FA more broadly, and we look forward to a continued partnership with the FDA through the Breakthrough Therapy designation and the CDRP program as we work to bring this potential treatment to patients as quickly as possible.”

The FDA decision was based on interim clinical data demonstrating that treatment with LX2006 was associated with clinically significant improvements in cardiac biomarkers and in cardiac and neurologic functional measures, with increased frataxin expression observed in all participants with cardiac biopsies at three months post treatment. To date, 17 participants have been treated across two trials: the Lexeo-sponsored SUNRISE-FA Phase 1/2 clinical trial (NCT05445323) and the Weill Cornell Medicine investigator-initiated Phase 1A trial (NCT05302271). Lexeo is currently enrolling a prospective natural history study, CLARITY-FA, which will serve as a concurrent external control arm for the registrational study. The Company expects to initiate the registrational study by early 2026 and is actively working with FDA to finalize the statistical analysis plan (SAP).

Breakthrough Therapy designation is intended to accelerate the development and review of investigational therapies that aim to treat serious or life-threatening diseases and where preliminary clinical evidence indicates that the therapy may demonstrate substantial improvement over available alternatives. This designation is in addition to Regenerative Medicine Advanced Therapy (RMAT) designation, Orphan Drug designation and Fast Track designation, all previously granted to LX2006 by the FDA. The CDRP program was created by the FDA to facilitate expedited CMC development of investigational therapies with expedited clinical development timeframes. This program increases communication between FDA and sponsors on CMC development specifically, with the goal of enabling earlier patient access to promising therapies in areas of high unmet need.

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 and the timing for receipt and announcement of data from its clinical trials, and the timing and likelihood of potential regulatory developments and approval. 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, 2024, filed with the SEC on March 24, 2025, Quarterly Report on Form 10-Q for the quarterly period ended March 31, 2025, filed with the SEC on May 12, 2025, as amended, 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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