Lexeo Therapeutics Announces Regulatory Update and Registrational Trial Design for LX2006 Gene Therapy in Friedreich Ataxia

June 15, 2026

SUNRISE-FA 2 study parameters include LVMI primary endpoint, 6-month topline efficacy analysis, inclusion criteria focused on abnormal baseline LVMI and open-label trial design

BLA supportive manufacturing strategy includes flexible process validation, including reduced PPQ manufacturing batches

SUNRISE-FA 2 initiation on track for Q2 2026, with first patient expected to be enrolled by end of June

Company to host webcast today at 8:00 AM ET

NEW YORK, June 15, 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 the Company has finalized the SUNRISE-FA 2 pivotal trial protocol and statistical analysis plan (SAP) intended to provide clinical evidence to support the submission of a Biologics License Application (BLA) to the U.S. Food and Drug Administration (FDA) for gene therapy candidate LX2006 under the accelerated approval pathway in 2028.

“We have reached a major milestone with the finalization of the SUNRISE -FA 2 pivotal study design, establishing a clear and rigorous path to evaluate LX2006 in Friedreich ataxia (FA) cardiomyopathy,” said Narinder Bhalla, M.D., Chief Medical Officer of Lexeo Therapeutics. “Patients living with FA, particularly those with cardiac involvement, have a significant unmet need for new treatment options and remain at the center of our efforts. This progress brings us one step closer to delivering a potential new therapy, and we remain focused on execution as we work to initiate the pivotal study and enroll the first patient by the end of the month.”

"FARA congratulates the Lexeo Therapeutics team on this important milestone and is deeply grateful for their commitment to advancing the first gene therapy program for Friedreich ataxia," said Jennifer Farmer, Chief Executive Officer of the Friedreich's Ataxia Research Alliance (FARA). "We also thank the participants and investigators in the SUNRISE-FA Phase I/II study, whose courage paved the way for this pivotal trial. We commend Lexeo for designing SUNRISE-FA 2 with scientific rigor while recognizing that a sham or placebo design is neither necessary nor appropriate in the context of gene therapy and adding a pediatrics arm to this study, putting patients first as we work urgently toward the first approved treatment for FA cardiomyopathy."

SUNRISE-FA 2 Pivotal Trial Protocol and SAP
SUNRISE-FA 2 is an open-label pivotal study in which 13 participants aged 16 years and older will receive a single, intravenous administration of high-dose LX2006 ($1.2x10^{12}$ vector genomes per kilogram), compared with 13 participants untreated with LX2006 (untreated control). The study does not include a placebo or sham procedure for participants in the untreated control arm.

The concurrent untreated control arm reflects key elements of an external natural history control while being implemented prospectively within the same protocol. This design incorporates FDA feedback aimed at reducing potential sources of bias and ensures consistency in study assessments and evaluation methods across both arms, without impacting key study parameters, including size and duration.

Key design elements include:

- **Primary endpoint:** Left ventricular mass index (LVMI), assessed via cardiac magnetic resonance imaging (MRI), with a topline efficacy readout at 6 months post-treatment.
  - The SAP is powered to detect an LVMI effect size of 15% or greater.
  - Informed by the clinically meaningful results observed to date in Phase I/II studies, the FDA has recommended removal of the cardiac frataxin protein expression co-primary endpoint, as it is no longer necessary to demonstrate proof of mechanism for LX2006.
- **Key secondary endpoints:** Measures of neurologic and cardiac outcomes and relevant biomarkers, including modified Friedreich Ataxia Rating Scale (mFARS), Kansas City Cardiomyopathy Questionnaire (KCCQ), high-sensitivity (hs) troponin-I, and lateral wall thickness.
- **Patient population:** The study will enroll participants with abnormal LVMI at baseline, defined as at least two standard deviations above the normal mean.
- **Statistical analysis plan:** Participants will be randomly allocated to receive LX2006 or to the untreated control arm. This random allocation approach is intended to eliminate patient selection bias between untreated and treated arms, and ensure balanced baseline characteristics, including LVMI.
- **Crossover eligibility**: Participants in the untreated control arm are eligible to cross over to receive LX2006 after 6 months and will be included in the 6-month efficacy analysis, as well as in all long-term follow-up assessments.
- **Pediatric cohorts:** Pediatric cohorts will be assessed for safety following dosing in participants aged 16 years and above.

The FDA has confirmed that no additional nonclinical bridging studies are required and Lexeo may use its optimized, high-yield Sf9-baculovirus final manufacturing process to initiate dosing in the SUNRISE-FA 2 pivotal study. Clinical drug product has been manufactured at commercial scale and is immediately available for patient dosing.

CLARITY-FA Natural History Study

CLARITY-FA is a natural history study that will provide supportive evidence on the untreated disease course for both accelerated and full approval. Enrollment is ongoing and progressing well. CLARITY-FA shares identical inclusion criteria with the SUNRISE-FA 2 pivotal study and patients enrolled are eligible to participate in SUNRISE-FA 2, with the first patient expected to enroll by the end of June.

Next Steps

- Lexeo remains in ongoing discussions with the FDA regarding the confirmatory evidence strategy, including the potential use of certain secondary endpoints at the 12-month time point in SUNRISE-FA 2 to support full approval, and will provide an update once finalized.
- Based on the study size and duration of SUNRISE-FA 2, as well as expected PPQ and process validation requirements, Lexeo expects a topline data readout in the second half of 2027 and a BLA submission under the accelerated approval pathway in the first half of 2028.

Corporate Webcast Details
Lexeo Therapeutics will host a webcast at 8:00 AM ET today, June 15, 2026. Analysts and investors can participate by accessing the webcast live on the News & Events page in the Investors section of Lexeo's website, www.lexeotx.com. The webcast will be archived on the company's website following the call.

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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