Lexeo Therapeutics Reports Third Quarter 2025 Financial Results and Operational Highlights

November 5, 2025

FDA open to pooling data from ongoing Phase I/II studies of LX2006 with data from pivotal trial, and to earlier co-primary endpoint assessment, to support a Biologics License Application

FDA approved comparability report between LX2006 HEK and Sf9 manufacturing processes in November 2025, endorsing use of Sf9 final commercial manufacturing process to begin dosing patients in upcoming pivotal study

LX2006 interim clinical data show clinically meaningful improvements across cardiac and neurologic measures of Friedreich ataxia, including left ventricular mass index and the modified Friedreich Ataxia Rating Scale

Completed enrollment of LX2020 HEROIC-PKP2 Phase I/II trial with ten participants dosed; interim data from low-dose cohort reported and additional clinical data from high-dose cohorts on track for January 2026

$154 million equity financing to support LX2006 registrational activities and further development of cardiac pipeline; in addition to Q3-25 end cash, cash equivalents and investments, expected to fund operations into 2028

NEW YORK, Nov. 05, 2025 (GLOBE NEWSWIRE) -- Lexeo Therapeutics, Inc. (Nasdaq: LXEO), a clinical stage genetic medicine company dedicated to pioneering novel treatments for cardiovascular diseases, today provided business updates across its portfolio and reported third quarter 2025 financial results.

“We continue to build significant momentum across our cardiac pipeline, and the recent financing strengthens our ability to execute on essential manufacturing and commercial activities for LX2006 as we look towards registrational readiness,” said R. Nolan Townsend, Chief Executive Officer of Lexeo Therapeutics. “Interim clinical data for LX2006 demonstrate meaningful benefit across measures of cardiac health and neurologic function, including improvements in the modified Friedreich Ataxia Rating Scale (mFARS), and we believe this therapy could represent a transformational step forward in the standard of care for FA. Furthermore, with enrollment complete in the HEROIC-PKP2 Phase I/II trial, we look forward to sharing new clinical data for LX2020 in January.”

Business and Program Updates

- **LX2006 in Friedreich Ataxia (FA):**
  - **Regulatory Progress**: In response to questions regarding the possibility of a faster path to a Biologics License Application (BLA), the FDA has indicated openness to a BLA submission for accelerated approval that includes clinical data from the ongoing Phase I/II studies of LX2006 pooled with new clinical data to be generated in the planned pivotal study. To enable pooling of these data to support licensure, Lexeo plans to submit enhanced manufacturing comparability data and additional nonclinical data. The FDA also previously agreed to evaluate the co-primary endpoint of left ventricular mass index (LVMI) at a time point earlier than 12 months. Lexeo continues to engage with the FDA on the pivotal trial protocol and enhanced comparability. There have been no changes to the previously disclosed alignment with FDA on key parameters related to the LX2006 planned registrational study to date.
  - **Interim Clinical Data:** In October 2025, Lexeo shared interim clinical data from both ongoing Phase I/II studies of LX2006, which continue to show encouraging safety and efficacy:
    - 18% mean improvement in LVMI at 6 months and 23% mean improvement at 12 months in participants with abnormal baseline LVMI (n=6), exceeding the 10% FDA-aligned efficacy threshold for the planned pivotal study
    - Improvement or stabilization in secondary cardiac biomarkers high-sensitivity troponin I and lateral wall thickness in 14 of 16 participants (n=16)
    - 2.0-point mean improvement in mFARS from baseline at latest visit across all participants with ≥6-months of follow-up (n=16)
    - Previously reported 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, exceeding FDA-aligned efficacy threshold for frataxin expression
    - Treatment with LX2006 has been generally well tolerated with no Grade 3+ SAEs to date, no clinically significant complement activation, and minimal, transient liver function test (LFT) elevations
  - **Analytical Comparability:** In November 2025, FDA approved the analytical comparability report establishing comparability between LX2006 HEK293 and Sf9 manufacturing processes. This approval endorses use of the optimized, Sf9 final commercial manufacturing process for LX2006 in the planned pivotal study and clears comparability requirements to begin dosing patients. In October 2025, Lexeo shared manufacturing data from this report demonstrating similar frataxin expression in vitro between the two processes, as well as the high quality of the Sf9 material with minimal residual DNA.
  - **Next Steps**: Lexeo plans to initiate a registrational study in the first half of 2026, pending finalization of the trial protocol in early 2026.

- **LX2020 in PKP2-ACM:**
  - **Dosing Update:** Enrollment is complete with ten participants dosed in the HEROIC-PKP2 Phase I/II clinical trial, including three participants in Cohort 1 at the low dose (2×10^{13} vg/kg), three participants in Cohort 2 at the high dose (6×10^{13} vg/kg), and four participants in dose-expansion Cohort 3 at the high dose (6×10^{13} vg/kg).
  - **Low-dose (Cohort 1) Interim Update**: In October 2025, Lexeo shared interim clinical data for the three participants dosed in Cohort 1 (n=2 at 12 months; n=1 at 9 months), assessing multiple cardiac parameters at latest visit relative to baseline:
    - Arrythmia burden: premature ventricular contractions (PVCs) were reduced in one of three participants and non-sustained ventricular tachycardia (NSVT) was reduced or stable in two of three participants
    - Electrical activity: QRS duration was normalized or stable in two of three participants and T-wave inversions were reduced in two of three participants
    - Cardiac function: left ventricular ejection fraction (LVEF) and right ventricular ejection fraction (RVEF) were stable in three of three participants
  - **Safety:** LX2020 continues to be generally well tolerated across participants with no clinically significant complement activation. One Grade 3 serious adverse event of sustained ventricular tachycardia (VT) was observed three months after dosing in a single participant in the high dose cohort and assessed as possibly treatment related. The participant was successfully treated with anti-arrhythmic medication and was discharged with no additional intervention required.
  - **Next Steps:** Lexeo expects to provide a substantive LX2020 data update in January. This data update is expected to include safety data for all ten participants dosed, clinical efficacy data for five high-dose participants at $\geq$6 months of follow up and three low-dose participants at $\geq$12 months of follow up, and cardiac biopsy data from five participants in high-dose Cohorts 2 and 3.
- **Closed $154 Million Equity Financing:** In October 2025, Lexeo announced the closing of an oversubscribed $154 million equity financing to further advance development of its cardiac pipeline and to support registrational readiness activities for LX2006.

Third Quarter Financial Results

- **Cash Position:** As of September 30, 2025, cash, cash equivalents, and investments in marketable securities were $122.8 million, excluding the $153.8 million of proceeds from the October 2025 public offering and concurrent PIPE which Lexeo believes will be sufficient to fund operations into 2028.
- **Research and Development Expenses:** Research and Development expenses were $15.7 million for the three months ended September 30, 2025, compared to $23.4 million for the three months ended September 30, 2024.
- **General and Administrative Expenses:** General and Administrative expenses were $6.0 million for the three months ended September 30, 2025, compared to $8.1 million for the three months ended September 30, 2024.
- **Net Loss:** Net loss was $20.3 million or $0.33 per share (basic and diluted) for the three months ended September 30, 2025, compared to $29.5 million or $0.89 per share (basic and diluted) for the three months ended September 30, 2024.

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, the timing and likelihood of potential regulatory developments and approval, expectations regarding the time period over which Lexeo's capital resources will be sufficient to fund its anticipated operations and estimates regarding Lexeo's financial condition. 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 Quarterly Report on Form 10-Q for the quarterly period ended June 30, 2025, filed with the SEC on August 14, 2025, 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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Lexeo Therapeutics, Inc.
Selected Financial Information
(Unaudited, in thousands, except share and per share amounts)

Condensed Statement of Operations

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2025	**2024**	**2025**	**2024**
Operating expenses				
Research and development	$ 15,695	$ 23,423	$ 47,587	$ 55,725
General and administrative	5,953	8,120	38,554	22,659
Total operating expenses	21,648	31,543	86,141	78,384
Operating loss	(21,648)	(31,543)	(86,141)	(78,384)
Other income and expense				
Gain on long-term investment	-	-	3,390	-
Other income (expense), net	(9)	(3)	(27)	(9)
Interest income	1,456	2,092	3,917	6,091
Amortization of premium on investments in U.S. Treasury securities	(60)	-	(106)	-
Total other income and expense	1,365	2,054	7,099	5,975
Loss from operations before income taxes	(20,283)	(29,489)	(79,042)	(72,409)
Income taxes	-	-	-	-
Net loss	$ (20,283)	$ (29,489)	$ (79,042)	$ (72,409)
Net loss per common share, basic and diluted	$ (0.33)	$ (0.89)	$ (1.72)	$ (2.31)
Weighted average number of shares outstanding used in computation of net loss per common share, basic and diluted	60,980,867	33,063,153	45,991,572	31,354,821

Condensed Balance Sheet Data

	September 30, 2025	December 31, 2024
Cash, cash equivalents, and investments in U.S. Treasury securities	$ 122,764	$ 128,530
Total assets	143,844	146,942
Total liabilities	23,013	30,100
Total stockholders' equity	120,831	116,842