Lexeo Therapeutics Announces Positive Interim Phase I/II Data and Regulatory Update for LX2020 in PKP2 Arrhythmogenic Cardiomyopathy and Reports Third Quarter 2026 Financial Results

November 16, 2026

Participants in high-dose cohorts achieved 80% mean reduction in non-sustained ventricular tachycardia and 44% mean reduction in premature ventricular contractions at latest assessment

LX2020 generally well tolerated across 10 participants with no clinically significant complement activation

U.S. Food and Drug Administration open to guideline-defined non-sustained ventricular tachycardia as a primary surrogate endpoint to support potential accelerated approval of LX2020

Cash, cash equivalents and investments of $219.1 million expected to provide operational runway into 2028

Company to host webcast today at 8:00 AM ET

NEW YORK, Nov. 16, 2026 (GLOBE NEWSWIRE) -- Lexeo Therapeutics, Inc. (Nasdaq: LXEO), a clinical stage company focused on reshaping the path of genetic diseases with high unmet need, today announced positive interim data from the ongoing HEROIC-PKP2 Phase I/II clinical trial of LX2020 for the treatment of PKP2 arrhythmogenic cardiomyopathy (PKP2-ACM). Across ten participants, including nine with at least 12 months of follow-up, treatment with LX2020 was generally well tolerated and associated with clinically meaningful reductions in arrhythmia burden, including non-sustained ventricular tachycardia (NSVT) and premature ventricular contractions (PVC), as well as improvement in measures of right ventricular function and patient-reported symptoms. Lexeo is now designing a pivotal study with NSVT as a primary surrogate endpoint to support potential accelerated approval. The final study design including the statistical analysis plan and confirmatory strategy remains subject to future discussions with the FDA.

“These data further strengthen our confidence in LX2020 and its potential to address the underlying biology of PKP2-ACM,” said R. Nolan Townsend, Chief Executive Officer of Lexeo Therapeutics. “The magnitude and consistency of NSVT reduction far exceed what available therapies have historically achieved, underscoring how LX2020 could meaningfully improve outcomes for individuals with PKP2-ACM. Our regulatory discussions also provide important clarity on the path forward, including the FDA’s openness to the use of NSVT as a primary endpoint in a planned pivotal study given the well-established association between NSVT and sustained, life-threatening arrhythmic risk.”

“For families living with PKP2 arrhythmogenic cardiomyopathy, the risks and uncertainties reach far beyond the clinic and into every part of daily life,” said Walker Frahm, Chief Executive Officer of the Foundation for Inherited Arrhythmias (FIA). “Our community has waited a long time for treatments that might address the underlying cause of this disease. Early results like these give ACM families reason to hope for better, more targeted care. We look forward to larger studies that build on these findings.”

LX2020 Interim Update

Ten participants have been 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) and seven participants in Cohorts 2 and 3 at the high dose (6×10^{13} vg/kg). As of the October 15, 2026 data cutoff date, nine of ten participants reached at least 12 months of follow-up, with the remaining participant reaching nine months of follow-up. Safety and efficacy data are summarized for all ten participants at latest assessment.

Clinical Data

- 80% mean reduction in NSVT at latest assessment relative to baseline in high-dose cohorts (n=7), with deepening reduction in mean NSVT burden over time; six of seven participants demonstrated improvement at latest assessment
- 44% mean reduction in PVC burden at latest assessment relative to baseline in high-dose cohorts (n=7); five of seven participants demonstrated improvement at latest assessment
- Five of seven participants in high-dose cohorts reported symptom improvement at latest visit relative to baseline on the Patient Global Impression of Change (PGIC) scale, a patient-reported outcome measure
- Preliminary evidence of stabilization or improvement in cardiac function assessed by cardiac MRI in the majority of participants in high-dose cohorts
- Cardiac biopsy findings demonstrated dose-dependent increases in LX2020 transduction, mean exogenous PKP2 mRNA expression and mean PKP2 protein expression

Safety

- LX2020 generally well tolerated across ten participants dosed
- No clinically significant complement activation was observed and no participant discontinued the study
- Previously disclosed elevations in liver function tests (LFT) were observed in the high-dose cohorts and were treated successfully per the trial protocol with no complications or hospitalizations
- One previously disclosed Grade 3 serious adverse event of ventricular tachycardia (VT) was observed three months after

dosing. This event is consistent with the natural course of PKP2-ACM and its known clinical manifestations. The participant was successfully treated with anti-arrhythmic medication and discharged with no additional intervention required

Regulatory Update and Next Steps

- The FDA is open to use of guideline-defined NSVT as a primary surrogate endpoint in an accelerated approval pivotal study
- Finalization of pivotal study design, statistical analysis plan and confirmatory evidence strategy expected in 1H 2027
- Lexeo expects to initiate a pivotal study supporting accelerated approval in 2H 2027

Q3 2026 Earnings Update
Today, Lexeo also reported financial results for the third quarter of 2026:

- **Cash Position:** As of September 30, 2026, cash, cash equivalents, and investments in marketable securities were $219.1 million, which Lexeo believes will be sufficient to fund operations into 2028
- **Research & Development Expenses:** Research and Development expenses were $34.5 million for the three months ended September 30, 2026, compared to $15.7 million for the three months ended September 30, 2025. The majority of the increase was driven by one-time costs including the acquisition of Mantle Therapeutics, which closed in September 2026, as well as a development milestone that was achieved in the third quarter of 2026 related to LX2006 pivotal study initiation
- **General & Administrative Expenses:** General and Administrative expenses were $5.9 million for the three months ended September 30, 2026, compared to $6.0 million for the three months ended September 30, 2025
- **Net Loss:** Net loss was $38.3 million or $0.43 per share (basic and diluted) for the three months ended September 30, 2026, compared to $20.3 million or $0.33 per share (basic and diluted) for the three months ended September 30, 2025

Lexeo also announced the appointment of Toby Schilke as an independent director to the Board of Directors. Most recently, Mr. Schilke served as Chief Financial Officer of Crinetics Pharmaceuticals. He previously served as Chief Financial Officer at Revance Therapeutics and Achaogen. Earlier in his career, he held leadership roles across corporate development, commercial finance, marketing, and global operations at Roche and Genentech.

Corporate Webcast Details
Lexeo Therapeutics will host a webcast at 8:00 AM ET today, November 16, 2026. Analysts and investors can participate by accessing the webcast live on the Events & Presentations 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 LX2020
LX2020 is an AAV-based gene therapy candidate for the treatment of plakophilin-2 arrhythmogenic cardiomyopathy (PKP2-ACM). Mutations in the *PKP2* gene are the most common genetic cause of ACM, responsible for approximately 50% of cases and estimated to affect approximately 60,000 people in the United States. PKP2 deficiency in ACM can lead to myocardial cell death, fibrosis, heart dysfunction, rhythm abnormalities, and sudden cardiac death. LX2020 is designed to systemically deliver a functional, full-length *PKP2* gene within an adeno-associated viral capsid, AAVrh10, to cardiomyocytes to restore the desmosomal complex and cell-to-cell adhesion. LX2020 is being evaluated in the single-arm, open-label, multi-center HEROIC-PKP2 Phase I/II clinical trial (NCT06109181). LX2020 has been granted Regenerative Medicine Advanced Therapy, Orphan Drug and Fast Track designations by the FDA.

About Lexeo Therapeutics
Lexeo Therapeutics is a New York City-based, clinical stage company dedicated to reshaping the path of genetic disease. By advancing pioneering science, Lexeo seeks to set a new standard in the treatment of cardiovascular and neurological genetic diseases, charting the path to patient outcomes once thought out of reach. The Company is advancing a portfolio of therapeutic candidates designed to address the underlying genetic causes of disease, including LX2006 for Friedreich ataxia (FA), LX2020 for PKP2-ACM, 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 anticipated benefits of its current product candidates, the timing and likelihood of potential regulatory developments, trial design changes and approval, and 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: expectations regarding the initiation, progress, and expected results of Lexeo's preclinical studies, clinical trials and research and development programs, including the outcome of ongoing discussions with the FDA regarding the design of the pivotal study to support an application for accelerated approval of LX2020, statistical analysis plan and confirmatory evidence strategy; 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, 2026, filed with the SEC on August 12, 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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Lexeo Therapeutics, Inc.
Selected Financial Information
(Unaudited, in thousands, except share and per share amounts)

Statements of Operations

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2026	2025	2026	2025
Operating expenses				
Research and development	$ 34,496	$ 15,695	$ 69,226	$ 47,587
General and administrative	5,903	5,953	21,526	38,554
Total operating expenses	40,399	21,648	90,752	86,141
Operating loss	(40,399)	(21,648)	(90,752)	(86,141)
Other income and expense				
Gain on long-term investment	-	-	-	3,390
Other expense, net	(9)	(9)	(16)	(27)
Interest expense	(12)	(22)	(43)	(75)
Interest income	2,160	1,456	6,467	3,917
Amortization of premium on investments in U.S. Treasury securities, net	(40)	(60)	(19)	(106)
Total other income and expense	2,099	1,365	6,389	7,099
Loss from operations before income taxes	(38,300)	(20,283)	(84,363)	(79,042)
Income taxes	-	-	-	-
Net loss	$ (38,300)	$ (20,283)	$ (84,363)	$ (79,042)
Net loss per common share, basic and diluted	$ (0.43)	$ (0.33)	$ (0.99)	$ (1.72)
Weighted average number of shares outstanding used in computation of net loss per common share, basic and diluted	88,681,360	60,980,867	85,191,438	45,991,572

Balance Sheet Data

	September 30, 2026	December 31, 2025
Cash, cash equivalents, and investments in U.S. Treasury securities	$ 219,134	$ 246,568
Total assets	243,916	268,688
Total liabilities	27,225	22,019
Total stockholders' equity	216,691	246,669