Lexeo Therapeutics Reports Fourth Quarter and Full Year 2024 Financial Results and Operational Highlights

March 24, 2025

Additional alignment with FDA on LX2006 planned pivotal study including protein expression co-primary endpoint: based on improvements in LVMI across participants with abnormal LVMI at baseline, frataxin expression to be evaluated for any increase from baseline rather than numerical threshold

Interim update from cohort 1 of LX2020 HEROIC-PKP2 Phase 1/2 trial: observed 71% and 115% increases in PKP2 protein expression in first two post-treatment biopsies; first participant evaluated 6-months after dosing experienced 67% reduction in premature ventricular contractions (PVCs)

Completed enrollment of cohort 2 of LX2020 HEROIC-PKP2 Phase 1/2 trial; interim clinical data update expected in second half of 2025

LX2020 has been generally well tolerated with no treatment-related serious adverse events to date

Cash, cash equivalents and investments of $128.5 million expected to provide operational runway into 2027

NEW YORK, March 24, 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 fourth quarter and full year 2024 financial results.

“We are pleased to share further regulatory clarity for LX2006 for the treatment of Friedreich ataxia (FA) cardiomyopathy, and we appreciate the continued partnership from the FDA on an accelerated approval pathway to support adults and children living with this devastating condition,” said R. Nolan Townsend, Chief Executive Officer of Lexeo Therapeutics. “We are also encouraged by the favorable safety profile and early data observed in participants dosed with LX2020 to date. We look forward to sharing additional clinical updates later in 2025 now that the second cohort of the LX2020 HEROIC-PKP2 Phase 1/2 trial in arrhythmogenic cardiomyopathy is fully enrolled.”

Business and Program Updates
LX2006 for the Treatment of FA Cardiomyopathy:

- **Regulatory Update:** Further alignment on elements of the accelerated development pathway following a Type B RMAT meeting with the U.S. Food and Drug Administration (FDA):
  - Frataxin expression to be evaluated for any increase from baseline rather than numerical threshold, based on improvements to date in LVMI across participants with abnormal LVMI at baseline
  - Inclusion of pediatric cohorts, both adolescents and children, in planned pivotal study
  - Use of prospective natural history data as external control in planned pivotal study
  - Final dose selection and remaining elements of registrational trial alignment expected in 2025

In light of these regulatory updates and anticipated timelines for assay validation, the Company expects to measure frataxin protein expression using liquid chromatography mass spectrometry (LCMS) in the planned registrational trial.

- **Mid-Year Clinical Update Expected to Include:**
  - Safety and tolerability data for all participants dosed across both the SUNRISE-FA and Weill Cornell clinical trials (at least 16 participants, including 6 participants with abnormal LVMI at baseline)
  - Pre- and post-treatment cardiac frataxin protein expression measured via LCMS for all four participants at the highest dose ($1.2x10^{12}$ vg/kg, Cohort 3)
  - Clinical biomarker data, including left ventricular mass index (LVMI), left ventricular wall thickness and high-sensitivity troponin I, for participants with ≥6-months of follow up
  - Functional and patient-reported outcome data for participants with ≥6-months of follow up
- **Safety:** LX2006 continues to be generally well tolerated with no new treatment-related serious adverse events to report

LX2020 for the Treatment of PKP2-ACM:

- **Cohort 1 Interim Update:** Post-treatment cardiac biopsies from two participants in cohort 1 showed increases in PKP2 protein expression from baseline; the third cohort 1 participant elected not to undergo a post-treatment biopsy
  - Observed increases in exogenous mRNA and 71% and 115% increases in PKP2 protein levels from baseline
  - First participant evaluated 6-months post treatment experienced 67% reduction in PVCs from baseline (from 861 to 284) and resolution of non-specific intraventricular block (normalization of QRS duration)
- **Enrollment Update:** Completed enrollment of cohort 2 of LX2020 HEROIC-PKP2 (n=3), interim clinical data update expected in second half of 2025
- **Safety:** LX2020 has been generally well tolerated with no treatment-related serious adverse events to date across both dose cohorts
- **Regulatory Update:** In March 2025 the European Commission granted orphan medicinal product designation for LX2020 for the treatment of PKP2-ACM

Fourth Quarter and Full Year Financial Results

- **Cash Position:** As of December 31, 2024, cash, cash equivalents, and investments were $128.5 million, which Lexeo believes will be sufficient to fund operations into 2027.
- **R&D Expenses:** R&D expenses were $18.4 million for the three months ended December 31, 2024, compared to $8.2 million for the three months ended December 31, 2023. R&D expenses were $74.1 million for the year ended December 31, 2024, compared to $53.1 million for the year ended December 31, 2023.
- **G&A Expenses:** G&A expenses were $9.0 million for the three months ended December 31, 2024, compared to $6.8 million for the three months ended December 31, 2023. G&A expenses were $31.7 million for the year ended December 31, 2024, compared to $15.4 million for the year ended December 31, 2023.
- **Net Loss:** Net loss was $25.9 million or $0.78 per share (basic and diluted) for the three months ended December 31, 2024, compared to $14.2 million or $0.86 per share (basic and diluted) for the three months ended December 31, 2023. Net loss was $98.3 million or $3.09 per share (basic and diluted) for the year ended December 31, 2024, compared to $66.4 million or $12.40 per share (basic and diluted) for the year ended December 31, 2023.

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 for the treatment of Friedreich ataxia (FA) cardiomyopathy, LX2020 for the treatment of plakophilin-2 (PKP2) arrhythmogenic cardiomyopathy, and other 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 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: 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, 2023, filed with the SEC on March 11, 2024, Quarterly Report on Form 10-Q for the quarterly period ended September 30, 2024, filed with the SEC on November 13, 2024, and subsequent future filings Lexeo may make with the SEC. Additional information will also be set forth in Lexeo's Annual Report on Form 10-K for the year ended December 31, 2024. 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.

Media Response:
Media@lexeotx.com

Investor Response:
Carlo Tanzi, Ph.D.
ctanzi@kendallir.com

Lexeo Therapeutics, Inc.
Selected Financial Information
(in thousands, except share and per share amounts)

Statements of Operations

	Three Months Ended December 31,		Year Ended December 31,	
	2024	2023	2024	2023
	(unaudited)	(unaudited)		
Operating expenses				
Research and development	$ 18,366	$ 8,210	$ 74,091	$ 53,130
General and administrative	9,016	6,764	31,675	15,383
Total operating expenses	27,382	14,974	105,766	68,513
Operating loss	(27,382)	(14,974)	(105,766)	(68,513)
Other income and expense				
Loss on fair value adjustment to convertible SAFE Note	-	(258)	-	(530)
Other income (expense), net	-	(8)	(9)	(13)
Interest expense	(30)	(51)	(137)	(205)
Interest income	1,465	1,103	7,556	2,867
Accretion of discount on investments	23	-	23	-
Total other income and expense	1,458	786	7,433	2,119
Loss from operations before income taxes	(25,924)	(14,188)	(98,333)	(66,394)

Income taxes	-	-	-	-
Net loss	$ (25,924)	$ (14,188)	$ (98,333)	$ (66,394)
Net loss per common share, basic and diluted	$ (0.78)	$ (0.86)	$ (3.09)	$ (12.40)
Weighted average number of shares outstanding used in computation of net loss per common share, basic and diluted	33,076,094	16,438,237	31,787,491	5,354,368

Balance Sheet Data

	December 31, 2024	December 31, 2023
Cash, cash equivalents, and investments	$ 128,530	$ 121,466
Total assets	146,942	139,807
Total liabilities	30,100	26,272
Total stockholders' equity	116,842	113,535