Lexeo Therapeutics Receives FDA Regenerative Medicine Advanced Therapy (RMAT) Designation for LX2020 for the Treatment of PKP2 Arrhythmogenic Cardiomyopathy

August 5, 2026

RMAT designation granted based on recent interim clinical data from ongoing Phase I/II clinical trial evaluating LX2020 for PKP2-ACM

LX2020 now holds RMAT, Orphan Drug and Fast Track designations by the FDA

NEW YORK, Aug. 05, 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 U.S. Food and Drug Administration (FDA) has granted Regenerative Medicine Advanced Therapy (RMAT) designation to LX2020, the Company's investigational AAV-based gene therapy candidate for the treatment of PKP2-associated arrhythmogenic cardiomyopathy (PKP2-ACM). RMAT designation for LX2020 is based on recent interim clinical data from the ongoing HEROIC-PKP2 Phase I/II clinical trial in PKP2-ACM.

"Receiving RMAT designation for LX2020 is an important regulatory milestone that underscores the strength of the emerging clinical evidence from our HEROIC-PKP2 trial and the potential of LX2020 to address the underlying genetic cause of PKP2-ACM," said Narinder Bhalla, M.D., Chief Medical Officer of Lexeo Therapeutics. "With no approved disease-modifying treatments currently available for patients living with this serious, progressive cardiovascular disease, this designation provides a valuable opportunity for early and ongoing engagement with the FDA as we work to advance LX2020 as a potential one-time gene therapy designed to address this significant unmet need. We look forward to sharing additional clinical and regulatory updates before the end of the year."

RMAT designation is an FDA expedited program intended to facilitate the development and review of regenerative medicine therapies for serious conditions where preliminary clinical evidence indicates the potential to address unmet medical needs. Along with Orphan Drug and Fast Track designations, RMAT designation provides enhanced opportunities for interaction with the FDA, including early and ongoing guidance regarding clinical development, manufacturing and potential regulatory pathways, and it may provide eligibility for accelerated approval, priority review and rolling review.

About LX2020
LX2020 is an AAV-based gene therapy candidate for the treatment of plakophilin-2-associated 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 RMAT, Orphan Drug and Fast Track designations by the FDA.

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, the timing for receipt and announcement of data from our 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: 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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