



LEXEO Therapeutics Announces Completion of First Cohort and Dosing in Second Cohort in SUNRISE-FA, a Phase 1/2 Clinical Trial of LX2006 for the Treatment of Friedreich's Ataxia Cardiomyopathy

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LX2006 is the first investigational gene therapy for the treatment of Friedreich's ataxia cardiomyopathy

Initial data from the first two cohorts, including myocardial protein expression and biomarkers measuring serum and cardiac structure and function, expected in the first half of 2024

NEW YORK, June 13, 2023 (GLOBE NEWSWIRE) -- [LEXEO Therapeutics](#) (LEXEO), a clinical-stage gene therapy company advancing adeno-associated virus (AAV)-based gene therapy candidates for genetically defined cardiovascular diseases and a genetically defined sub-group of Alzheimer's disease, announced today the completion of the first dose cohort and the dosing of the first patient in the second dose cohort in SUNRISE-FA, a Phase 1/2 clinical trial of LX2006 in patients with Friedreich's ataxia (FA) cardiomyopathy.

In the first dose cohort, LX2006 has been well tolerated with no unexpected events or toxicities observed. Following the Data Safety Monitoring Board recommendation to proceed, investigators have initiated dosing in the second cohort.

"New treatment approaches, like LEXEO's LX2006 gene therapy candidate, are critical for individuals and caregivers confronted with the debilitating realities of FA," said Friedreich's Ataxia Research Alliance Chief Executive Officer Jennifer Farmer. "Cardiomyopathy is the leading cause of death in individuals diagnosed with FA, so we are incredibly encouraged by the work that LEXEO has undertaken to try to address this life-threatening complication through gene therapy. We are grateful to all the individuals in the FA community who volunteer and participate in research studies and clinical trials."

"The advancement of SUNRISE-FA helps pave the way for a potential life-altering treatment for patients with FA cardiomyopathy. Because no approved therapy has been shown to treat the cardiac manifestations of FA, a significant unmet need persists for these patients," said R. Nolan Townsend, Chief Executive Officer of LEXEO Therapeutics. "We look forward to continuing to progress this program with data readouts expected in the first half of 2024."

SUNRISE-FA is a multicenter, 52-week, dose-ascending, open-label trial evaluating the safety and tolerability, as well as preliminary efficacy, of LX2006 in patients who have FA cardiomyopathy. LX2006 is administered as a one-time intravenous infusion to patients in at least two ascending-dose cohorts. Long-term safety and efficacy will be evaluated for an additional four years following completion of the initial trial, resulting in data from a total of five years post-LX2006 treatment.

For additional information about the trial, visit www.clinicaltrials.gov using the study identifier [NCT05445323](#).

About LX2006

LX2006 is an AAV-based gene therapy candidate delivered intravenously for the treatment of FA cardiomyopathy, the most common cause of mortality in patients with FA affecting approximately 5,600 patients in the United States. LX2006 is designed to target the cardiac manifestations of FA by delivering a functional frataxin gene to promote the expression of the frataxin protein and restore mitochondrial function in myocardial cells. In preclinical studies, LX2006 reversed the cardiac abnormalities in FA disease models and showed improvement in cardiac function and survival while demonstrating a favorable safety profile. The FDA has granted Rare Pediatric Disease designation and Orphan Drug designation to LX2006 for the treatment of Friedreich's ataxia. SUNRISE-FA is an ongoing Phase 1/2 clinical trial evaluating the safety and efficacy of LX2006.

About LEXEO Therapeutics

LEXEO Therapeutics is a New York City-based, clinical-stage gene therapy company focused on addressing some of the most devastating genetically defined cardiovascular diseases and a genetically defined sub-group of Alzheimer's disease. LEXEO's foundational science stems from partnerships and exclusive licenses with leading academic laboratories at Weill Cornell Medicine and the University of California, San Diego. LEXEO is advancing a deep and diverse pipeline of AAV-based gene therapy candidates for diseases affecting both larger-rare and prevalent patient populations and is led by pioneers and experts with decades of collective experience in genetic medicines, rare disease drug development, manufacturing, and commercialization. For more information, please visit www.lexeotx.com or LinkedIn.

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