LEXEO Therapeutics Announces FDA Clearance of Investigational New Drug Application for LX2006, an AAV-based Gene Therapy Candidate for Friedreich's Ataxia Cardiomyopathy

- Phase 1/2 clinical trial expected to initiate in mid-2022 -

- LX2006 is the first clinical-stage program from LEXEO's cardiovascular pipeline and the third clinical-stage gene therapy candidate across its pipeline -

NEW YORK – February 16, 2022 (GLOBE NEWSWIRE) – LEXEO Therapeutics (LEXEO), a clinical-stage gene therapy company advancing a diverse pipeline of adeno-associated virus (AAV)-based gene therapy candidates for genetically defined cardiovascular and central nervous system diseases, today announced that the U.S. Food and Drug Administration (FDA) has cleared its Investigational New Drug (IND) application for LX2006. LX2006 is an AAV-based gene therapy candidate designed to intravenously deliver a functional frataxin gene, for the treatment of Friedreich's ataxia cardiomyopathy (FA cardiomyopathy). LEXEO plans to initiate this open-label, dose-escalation Phase 1/2 clinical trial of LX2006 in patients with FA cardiomyopathy in the middle of 2022.

"This IND clearance represents an important milestone for LEXEO. Representing our third clinical-stage program overall, LX2006 is also the first disease-modifying, clinical-stage gene therapy treatment for Friedreich's ataxia and the first clinical-stage program from our cardiovascular gene therapy pipeline," said R. Nolan Townsend, Chief Executive Officer of LEXEO.

"The potential of LX2006 is supported by our robust preclinical studies showing that LX2006 was able to significantly reverse the cardiac manifestations of the disease, which is the leading cause of death in individuals with Friedreich's ataxia. We anticipate initiating this Phase 1/2 clinical trial in the middle of this year and look forward to bringing the first potentially disease-modifying gene therapy to patients suffering from FA cardiomyopathy," commented Jay A. Barth, M.D., Executive Vice President and Chief Medical Officer of LEXEO.

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 Friedreich's ataxia. 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.

The Phase 1/2 study is a 52-week, dose-ascending, open-label trial of LX2006 in patients who have FA cardiomyopathy. LX2006 will be administered as a one-time intravenous infusion to patients in two ascending-dose cohorts of five patients each.

About Friedreich's Ataxia

Friedreich's ataxia is a genetic, progressive, degenerative multi-system disorder with a prevalence of 1:40,000, or approximately 8,000 people in the United States. It is estimated that roughly 70% or 5,600 of these patients will develop FA cardiomyopathy. FA is caused by a mutation in the frataxin gene that disrupts the normal production of the protein frataxin, which is critical to the function of mitochondria

in cells, in particular affecting neurons and myocardial cells, and is important in the maintenance of cardiac function. It is inherited in an autosomal recessive manner and symptoms usually begin in childhood. Deficiency of frataxin leads to damage to peripheral nerves and the parts of the brain that control movement and balance, leading to neurological symptoms that include impaired muscle coordination, or ataxia, which worsen over time. As the disease progresses, patients typically experience various cardiac abnormalities, which include fibrosis, thickening of the heart muscle, arrhythmias, and heart failure, and are the cause of death in nearly two-thirds of FA patients.

About LEXEO Therapeutics

LEXEO is a New York City-based clinical-stage gene therapy company focused on addressing some of the most devastating genetically defined cardiovascular and central nervous system diseases affecting both larger-rare and prevalent patient populations. The company's foundational science stems from partnerships and exclusive licenses with leading academic laboratories at Weill Cornell Medicine and University of California, San Diego, two preeminent institutions on the cutting edge of gene therapy research. LEXEO is advancing a deep and diverse pipeline of AAV-based gene therapies in rare cardiovascular diseases, *APOE4*-associated Alzheimer's disease, and CLN2 Batten disease, 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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