



LEXEO Therapeutics Announces License Agreement and Consolidation of Pre-clinical Data Package to Support Cardiac Friedreich's Ataxia Gene Therapy Program (LX2006)

March 1, 2021

LEXEO completes license of all intellectual property rights and pre-clinical data associated with Adverum Biotechnologies' investigational Friedreich's ataxia program

Pre-clinical research licensed from academic partners and data from Adverum's program now consolidated to support the advancement of LX2006; Phase 1 clinical trial expected to initiate in 2021

NEW YORK, March 01, 2021 (GLOBE NEWSWIRE) -- [LEXEO Therapeutics](#), a clinical-stage gene therapy company, today announced it has licensed worldwide intellectual property rights and pre-clinical data from Adverum Biotechnologies to its Friedreich's ataxia gene therapy program. With exclusive rights to data from seven pre-clinical studies now combined, LEXEO will advance LX2006 through final IND-enabling studies and into a planned Phase 1 clinical trial in 2021.

LX2006 is an adeno-associated virus (AAV)-mediated treatment delivered through intravenous administration to address the Friedreich's ataxia cardiac disease pathology, the most common cause of mortality in people with Friedreich's ataxia. This program is supported by seven distinct pre-clinical studies conducted at preeminent scientific institutions in France and the United States examining multiple vector constructs, dose ranges and routes of administration, all contributing to the optimization of the current LX2006 program construct that will be taken forward into the clinic this year. In several pre-clinical studies, LX2006 has [demonstrated efficacy](#) in reversing the abnormal cardiac phenotype in Friedreich's ataxia disease models by transferring a normal frataxin gene to the heart.

"Our agreement with Adverum, adding to studies conducted by other academic partners, greatly strengthens our pre-clinical data package supporting LX2006, our gene therapy program addressing cardiomyopathy associated with Friedreich's ataxia," said R. Nolan Townsend, Chief Executive Officer of LEXEO Therapeutics. "Cardiomyopathy is the cause of mortality in up to 70% of Friedreich's ataxia patients. Our goal is to address this challenging component of Friedreich's ataxia with the first potentially disease-modifying therapy for this underserved patient population."

About Friedreich's Ataxia

Friedreich's ataxia (FA) is a rare, degenerative multi-system disorder affecting approximately 1 in 50,000 people in the United States. FA is caused by a gene mutation that disrupts the normal production of the protein frataxin, critical to the function of mitochondria (the energy producing factories) in a cell. FA is inherited in an autosomal recessive manner, usually begins in childhood, and leads to impaired muscle coordination (ataxia) that worsens over time, typically progressing to serious heart conditions, including hypertrophic cardiomyopathy and arrhythmias. FA is also associated with vision impairment, hearing loss, scoliosis, diabetes and slurred speech. Friedreich's ataxia can shorten life expectancy, and heart failure is the most common cause of death. LX2006 is an IV-administered, AAV-mediated frataxin gene therapy treatment focused on the cardiac pathology of FA. LEXEO Therapeutics is completing IND-enabling pre-clinical studies and expects to initiate a Phase 1 trial in 2021.

About LEXEO Therapeutics, Inc.

LEXEO Therapeutics is a New York City-based, fully integrated biotechnology company currently headquartered at the Alexandria Center® for Life Science that aims to apply the transformational science of gene therapy to address some of the world's most devastating genetic and acquired diseases. LEXEO Therapeutics' pipeline consists of adeno-associated virus (AAV)-mediated therapies primarily developed at Weill Cornell Medicine's Department of Genetic Medicine. Beyond LEXEO Therapeutics' lead programs – which are focused on both rare and non-rare monogenic (single gene mutation) diseases – the company's pre-clinical pipeline spans monogenic diseases, as well as hereditary and acquired diseases across a spectrum of patient population sizes and a range of unmet medical needs. Importantly, LEXEO Therapeutics will focus on advancing clinical programs through to commercialization, with the goal of maintaining an ongoing research collaboration with Weill Cornell Medicine's Department of Genetic Medicine to help advance the company's pre-clinical pipeline. For more information, please visit www.lexeotx.com or [LinkedIn](#).

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