LEXEO Therapeutics Receives Rare Pediatric Disease & Orphan Drug Designations for LX2006 for the Treatment of Friedreich's Ataxia

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Phase I/II clinical trial in patients with cardiomyopathy associated with Friedreich's ataxia expected to initiate in 2021

NEW YORK – June 30, 2021 (GLOBE NEWSWIRE) – LEXEO Therapeutics, a clinical-stage gene therapy company, today announced that the U.S. Food and Drug Administration (FDA) has granted Rare Pediatric Disease designation and Orphan Drug designation to LX2006 for the treatment of Friedreich's ataxia (FA). LX2006 is an IV-administered, adeno-associated virus (AAV)-mediated gene therapy encoding the human frataxin gene. The designations granted to LX2006 cover cardiac disease and broader symptoms associated with FA.

The FDA grants Rare Pediatric Disease designation for serious and life-threatening diseases that primarily affect children ages 18 years or younger and fewer than 200,000 people in the U.S. If a biologics license application (BLA) for LX2006 is approved, LEXEO may be eligible to receive a priority review voucher that may be sold or transferred.

The Orphan Drug designation is granted by the FDA to drugs or biologics intended to treat a rare disease that affects fewer than 200,000 people in the U.S. Programs with Orphan Drug status are eligible for various development incentives.

"Being granted both Rare Pediatric Disease and Orphan Drug designation shows the tremendous urgency for new, impactful therapeutic approaches such as LX2006 for people diagnosed with Friedreich's ataxia," said R. Nolan Townsend, Chief Executive Officer of LEXEO Therapeutics. "It is critical that we advance new disease-modifying therapies with the potential to transform the lives of FA patients, and we look forward to continuing our collaboration with the FDA as we advance LX2006 through clinical development."

FA is a rare, degenerative, multi-system disorder affecting approximately one in 50,000 people in the United States. It is an inherited condition caused by a gene mutation that disrupts the normal production of the protein frataxin, which functions in the mitochondria of the cell. It is inherited in an autosomal recessive manner, usually beginning in childhood and leads to impaired muscle coordination (ataxia) that worsens over time, typically progressing to serious heart conditions that can lead to heart failure, which is the most common cause of death in FA patients.

LEXEO plans to initiate a Phase I/II clinical trial of LX2006 in patients with cardiomyopathy associated with FA 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 preclinical 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 http://www.lexeotx.com/or LinkedIn.

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