



LEXEO Therapeutics Announces FDA Fast Track Designation Granted to LX1001 for the Treatment of APOE4 Associated Alzheimer's Disease

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LX1001 is the first investigational gene therapy being evaluated to address the underlying genetics of Alzheimer's disease

Initial Phase 1 clinical data expected in 2H 2021

NEW YORK, April 20, 2021 (GLOBE NEWSWIRE) -- [LEXEO Therapeutics](#), a clinical-stage gene therapy company, today announced that the U.S. Food and Drug Administration (FDA) has granted Fast Track designation to LX1001, the company's adeno-associated virus (AAV) mediated gene therapy program, for the potential treatment of apolipoprotein E4 (APOE4) associated Alzheimer's disease. LX1001 is a CNS-administered therapy being evaluated in an ongoing Phase 1 clinical trial.

"The Fast Track designation granted by the FDA will expedite the development and review of LX1001 and we look forward to working closely with the Agency moving forward as we continue to advance this transformational gene therapy," said R. Nolan Townsend, Chief Executive Officer of LEXEO Therapeutics. "While other treatments in development focus on the pathogenesis of Alzheimer's disease, LX1001 is the first investigational gene therapy designed to correct the underlying genetics of the disease thereby addressing the most significant risk factor for developing Alzheimer's disease."

Alzheimer's disease is the leading cause of late life mental failure in humans. APOE is the major transporter of cholesterol in the brain with prevailing evidence suggesting that it is a major genetic risk factor in the pathogenesis of the disease. Inheritance risk for APOE4 results in more frequent and earlier age of onset for developing Alzheimer's disease while inheritance of APOE2, a protective APOE gene variant, has the reverse effect. LX1001 is an AAV mediated gene therapy being developed to deliver the protective APOE2 gene to the central nervous system.

LEXEO is currently conducting an open label, dose-ranging Phase 1 clinical trial ([NCT0363400](#)) to evaluate LX1001 as a potential one-time treatment for early-stage Alzheimer's disease patients with mild cognitive impairment who are APOE4 homozygous. The study will assess the safety and toxicity of LX1001, and establish a maximum tolerable dose. The study is on schedule to complete dosing of 15 patients by the end of the year, and initial clinical data are expected in the second half of 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 www.lexeotx.com or LinkedIn.

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