LEXEO Therapeutics Launches with $85 Million Series A Financing to Develop Gene Therapies for Rare and Non-Rare Monogenic Diseases

Rare disease and gene therapy industry veterans Steven Altschuler, M.D., R. Nolan Townsend and Jay Barth, M.D., team up with gene therapy pioneer Ronald Crystal, M.D., to launch fully integrated gene therapy company

Financing led, structured and syndicated by Longitude Capital and Omega Funds

Comprehensive pipeline includes three clinical-stage gene therapy programs in monogenic diseases and up to 15 potential additional AAV gene therapy programs in monogenic and acquired diseases primarily developed at Weill Cornell Medicine

NEW YORK, Jan. 07, 2021 (GLOBE NEWSWIRE) -- LEXEO Therapeutics, a clinical-stage gene therapy company, debuted today with an oversubscribed $85 million Series A financing, led by Longitude Capital and Omega Funds, and joined by Lundbeckfonden Ventures, PBM Capital, Janus Henderson Investors, Invus, Woodline Partners LP, the Alzheimer's Drug Discovery Foundation[i] and Alexandria Venture Investments. Proceeds from the financing will help advance the company's three lead investigational programs, including: LX2006, an IV-administered therapy for cardiomyopathy associated with Friedreich's ataxia (Phase 1 start planned for 2021); LX1004, a CNS-administered therapy for CLN2 Batten disease (Phase 1/2 completed); and LX1001, a CNS-administered therapy for APOE4-associated Alzheimer's disease (Phase 1 ongoing).

"We are thrilled to launch today with a mission to advance LEXEO's promising clinical-stage pipeline of gene therapy treatments for patients diagnosed with some of society's most challenging diseases," said R. Nolan Townsend, Chief Executive Officer of LEXEO Therapeutics. "We have the pleasure of collaborating with Dr. Ronald Crystal – one of the industry's most accomplished pioneers in the gene therapy space – and, drawing on my years of rare disease leadership experience at Pfizer, will together build a world-class gene therapy organization driven by premier science that has the potential to address a range of therapeutic indications."

Founder and Chief Scientific Advisor Dr. Ronald Crystal is Professor and Chairman of Weill Cornell's Department of Genetic Medicine and Director of the Belfer Gene Therapy Core Facility. Dr. Crystal has more than 30 years of experience with adenovirus and adeno-associated virus vectors, from basic vector design through clinical development. Dr. Crystal has 14 approved gene therapy investigational new drug applications and has published more than 300 papers on gene therapy, with experience in CNS, cardiac, pulmonary and liver-mediated diseases.

"I am excited to work with LEXEO Therapeutics to move our extensive academic portfolio into clinical development and ultimately bring it to patients," said Dr. Crystal. "LEXEO's AAV-mediated gene therapy programs have the potential for broad applicability across a range of therapeutic indications, and in a single company pipeline present an opportunity for the natural evolution of gene therapy from rare genetic conditions to more common diseases."

LEXEO Therapeutics' Chairman, Dr. Steven Altschuler, is currently Managing Director at Ziff Capital Investments and was formerly Chairman of gene therapy biotech pioneer Spark Therapeutics, which was responsible for the first FDA-approved gene therapy, Luxturna®, and was acquired by Roche in 2019 for $4.3 billion.

"LEXEO's impressive management team, with Nolan's years of rare disease leadership experience at Pfizer, as well as its pioneering scientific founder and high-quality investor syndicate, will propel the development of the company's pipeline of promising and innovative programs," said Dr. Altschuler. "I am honored to have the opportunity to partner with Nolan and his team to build a leading gene therapy company."

LEXEO Therapeutics has also appointed Dr. Jay Barth as Executive Vice President and Chief Medical Officer to oversee Medical Affairs, Regulatory Affairs and Clinical Development. Dr. Barth was formerly the Chief Medical Officer at Amicus Therapeutics and Senior Vice President, Clinical Development, at PTC Therapeutics, where he oversaw all clinical development programs and the approval of Galafold® for Fabry disease at Amicus, as well as Translarna®, the first approved treatment for Duchenne muscular dystrophy at PTC Therapeutics.

Other members of LEXEO Therapeutics' Board of Directors include CEO R. Nolan Townsend, Sandip Agarwala of Longitude Capital, Bernard Davitian of Omega Funds and Mette Kirstine Agger of Lundbeckfonden Ventures.

Initial Indications

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 arrythmias. FA is also associated with vision impairment, hearing loss, scoliosis, diabetes and slurred speech. Friedreich's ataxia can shorten life expectancy, with heart failure the most common cause of death. Supported by de novo, soon to be published pre-clinical research, LX2006 is an IV-administered AAV-mediated frataxin gene therapy treatment focused on the cardiac pathology of FA. The company is completing IND-enabling pre-clinical studies and expects to initiate a Phase 1 trial in 2021.

CLN2 disease (late infantile Batten disease) is an autosomal recessive lysosomal storage disease with fewer than 1,000 cases worldwide, with typical onset in children between 2 and 4 years of age. The disease is caused by mutations in the CLN2 gene, resulting in progressive cognitive impairment, visual failure, seizures and deteriorating motor development. LX1004 is an AAV-meditated gene therapy treatment delivering CLN2 to the central nervous system. In December 2020, clinical data published in *Science Translational Medicine* found a single administration of AAV-mediated CLN2 gene therapy

(LX1004) slowed the progression of CLN2 disease in children. Treatment with LX1004 was well tolerated, with minimal serious adverse events in the acute/post-operative period (0-14 days) and over the 18-month study period (14 days – 18 months). With this Phase 1/2 study complete, the company plans to advance the program into a pivotal study in 2022.

Alzheimer's disease is the leading cause of late-onset dementia, characterized by progressive memory loss and cognitive decline in humans. APOE is a major cholesterol transporter and is in part linked to the pathogenesis of Alzheimer's disease due to development of amyloid plaques and tau-tangles in the brain. People who inherit APOE4 alleles are at significantly higher risk for developing Alzheimer's disease and at an earlier age of onset than people who inherit APOE3 or APOE2 alleles, which have normal and reduced risk of disease onset, respectively.[ii] LX1001 is an AAV-mediated gene therapy treatment delivering APOE2 to the central nervous system of people with two APOE4 alleles (homozygotes), via a CNS route of administration. A Phase 1 clinical study is ongoing.

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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[i] https://www.alzdiscovery.org/
[ii] https://www.ncbi.nlm.nih.gov/pmc/articles/PMC1350934/