LEXEO Therapeutics Announces Upcoming Data Presentations at the American Society of Gene and Cell Therapy (ASGCT) 2021 Virtual Annual Meeting

Data evaluating intravenous delivery of the AAVrh.10 viral vector show effectiveness in providing high levels of gene transfer to the heart for the potential treatment of rare cardiac diseases

Data on second generation investigational gene therapy demonstrate ability to silence the APOE4 gene for the potential treatment of Alzheimer's disease

Additional pre-clinical data on the company's gene therapy technology platform will also be presented

NEW YORK, April 28, 2021 (GLOBE NEWSWIRE) -- LEXEO Therapeutics, a clinical-stage gene therapy company, today announced upcoming presentations regarding its investigational gene therapy programs at the American Society of Gene and Cell Therapy (ASGCT) 24th Annual Meeting, taking place virtually from May 11-14, 2021.

Data from several abstracts will be presented, including:

Results evaluating intravenous (IV) delivery of the AAVrh.10 vector with genes coding for human frataxin protein, human α1-antitrypsin, or the luciferase reporter gene. These results demonstrate effective delivery of the AAVrh.10 vector to the heart.

Based on the high affinity of the AAVrh.10 vector for the myocardium, the muscular tissue of the heart, IV administration of this vector, a minimally invasive outpatient procedure, has demonstrated its potential as an effective delivery method for adeno-associated virus (AAV)-mediated gene therapy to potentially treat rare cardiac diseases. The AAVrh.10 vector is currently being evaluated by LEXEO in pre-clinical studies for the treatment of cardiomyopathy associated with Friedreich's ataxia (FA). Cardiomyopathy is the most common cause of mortality in patients with FA; it is the cause of death in nearly 70% of the patient population.

Title: High Affinity Cardiac Gene Transfer Mediated by Systemic Delivery of AAVrh.10 Vectors
Session: AAV Vectors - Preclinical and Proof-of-Concept Studies
Presenter: Bishnu P. De, Ph.D.
Presentation Date & Time: Tuesday, May 11, 2021, 8:00 - 10:00 a.m. ET

Results from a second generation investigational gene therapy program show effective delivery of APOE2, the gene associated with protection against the development of Alzheimer's disease, while also targeting and silencing APOE4, the gene that increases risk of developing the disease. A 40% reduction was observed in APOE4 expression, while APOE2 expression was maintained, suggesting APOE2 was resistant to microRNAs (miRNAs) silencing. The efficacy of gene silencing by miRNAs targeting APOE4 was evaluated by inserting miRNA into the AAV vector expression cassette.

Title: Second Generation $APOE2^{+}APOE4^{-}$ AAV-mediated Gene Therapy for APOE Homozygotes at Risk for Alzheimer's Disease
Session: AAV Vectors - Preclinical and Proof-of-Concept Studies
Presenter: Rachel Montel
Presentation Date & Time: Tuesday, May 11, 2021, 8:00 - 10:00 a.m. ET

Additional poster presentations highlighting LEXEO's platform technology include the following:

Title: siRNA Regulation of Erythropoietin Gene Therapy
Session: AAV Vectors - Preclinical and Proof-of-Concept Studies
Presenter: Fiona Hart
Presentation Date & Time: Tuesday, May 11, 2021, 8:00 - 10:00 a.m. ET

Title: Assessment of Whole Body Anti-capsid Neutralizing Immunity Using Quantitative I-124 Whole-Body Functional Positron Emission Tomography Imaging of Adeno-Associated Viral Vector Biodistribution in Nonhuman Primates
Session: AAV Vectors - Preclinical and Proof-of-Concept Studies
Presenter: Jonathan B. Rosenberg, Ph.D.
Presentation Date & Time: Tuesday, May 11, 2021, 8:00 - 10:00 a.m. ET

Title: Turning Off AAV-delivered Transgenes Using Artificial microRNAs
Session: Oligonucleotide Therapeutics
Presenter: Youjun Wu, Ph.D.
Presentation Date & Time: Tuesday, May 11, 2021, 8:00 - 10:00 a.m. ET

"We are encouraged by the diverse and robust data being presented at this year's ASGCT conference, highlighting important research progress achieved in collaboration with Weill Cornell Medicine's Department of Genetic Medicine across our gene therapy programs," said R. Nolan Townsend, Chief Executive Officer of LEXEO Therapeutics. "These data further support the development of our pre-clinical and clinical pipeline, and in particular, support our strategies to advance the cardiac and CNS programs, where we see significant potential for disease-modifying gene therapy treatments."

Dr. Ronald Crystal, Founder and Chief Scientific Advisor of LEXEO Therapeutics, and Chairman of the Department of Genetic Medicine at

Weill Cornell Medicine, commented, “These data are encouraging as they further validate the potential of LEXEO’s gene therapy platform to address a number of genetic diseases with no current disease-modifying treatment options available. In particular, these data show that we could help prevent high-risk individuals from developing Alzheimer’s disease by silencing the APOE4 gene while delivering the protective APOE2 gene. Additionally, data presented on the AAVrh.10 vector show the promise of this vector as an effective delivery approach to treat rare cardiac conditions and overcome safety challenges associated with high-dose AAV mediated gene therapy.”

LEXEO plans to initiate a Phase 1 clinical trial of LX2006 in patients with cardiomyopathy associated with FA and expects initial data from the Phase 1 trial of LX1001 in APOE4 associated Alzheimer’s disease in 2021.

These abstracts are available on the ASGCT Annual Meeting website, and posters can be accessed by conference participants through the ASGCT website on May 11, 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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