LEXEO Therapeutics Announces Data Presentations at the 26th American Society of Gene & Cell Therapy (ASGCT) Annual Meeting

Research targeting cardiac and neurological conditions to be featured across eight presentations at ASGCT Annual Meeting

NEW YORK – May 2, 2023 (GLOBE NEWSWIRE) – LEXEO Therapeutics (LEXEO), a clinical-stage gene therapy company advancing adeno-associated virus (AAV)-based gene therapy candidates for genetically defined cardiovascular and central nervous system (CNS) diseases, announced today that new data supporting its cardiac and neurological gene therapy programs will be presented at the 26th Annual Meeting of the American Society of Gene & Cell Therapy (ASGCT) taking place May 16-20, 2023.

"LEXEO's latest research continues to support our efforts to address difficult-to-treat conditions like cardiac arrhythmogenic right ventricular cardiomyopathy, Friedreich's ataxia and APOE4-associated Alzheimer's disease," said Nolan Townsend, Chief Executive Officer of LEXEO. "The findings from this research mark important advances across our pipeline and help shape a path forward to making these potential gene therapies a reality for the patients affected by these devastating conditions."

Eight abstracts, including an oral presentation, were selected to be presented at ASGCT.

Presentations include:

Title: LX2020 – An AAV-Based Gene Therapy Improves The Arrhythmogenic Right Ventricular Cardiomyopathy Phenotype In A Severe Mouse Model Harboring Human PKP2 Mutation
Presenter: Farah Sheikh, Ph.D., University of California San Diego School of Medicine*
Date/Time: Thursday, May 18, 5:00 – 5:15 p.m. PT
Location: Room 409 AB
Session Title: Breaking Gene Editing and Other Barriers for Cardiovascular and Pulmonary Diseases
Abstract Number: 205

- **Abstract highlights:** A preclinical study examined effects of LX2020 – a novel AAVrh.10 based gene therapy – in arrhythmogenic right ventricular cardiomyopathy (ARVC) by restoring the often-mutated desmosomal cell-cell junction gene, plakophilin-2 (PKP2). The efficacy of LX2020 was tested in mice engineered to harbor a human PKP2 splice site mutation, found in multiple ARVC populations, and that exhibited severe ARVC disease. Results showed restoration of cardiac PKP2 levels and prevention of cardiac desmosome and cell-cell junctional protein deficits, which translated to functional and survival improvements in adult PKP2 homozygous mutant mice. These data indicate that LX2020 overcomes cardiac PKP2 deficits in ARVC settings by driving human PKP2 expression and that this is sufficient to prevent and stabilize the progression of rapid ARVC disease in a novel PKP2 genetic mouse model.

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

Cardiac Posters

Title: Dose-dependent Cardiac Responses to Intravenous AAVrh.10hFXN Treatment of the MCK Murine Model of Friedreich's Ataxia
Presenter: Dolan Sondhi, Ph.D., Weill Cornell Medicine*
Date/Time: Wednesday, May 17, 12:00 – 2:00 p.m. PT
Abstract Number: 363

Title: Vector Biodistribution and Transgene Expression in the Heart Following Gene Transfer of AAVrh.10 vs. AAV9 Capsids
Presenter: Nithya Selvan, Ph.D., LEXEO Therapeutics
Date/Time: Thursday, May 18, 12:00 – 2:00 p.m. PT
Abstract Number: 1241

Title: Engineering of the AAVrh.10 Capsid for Cardiac Gene Transfer
Presenter: Slawomir Andrzejewski, Ph.D., Weill Cornell Medicine
Date/Time: Friday, May 19, 12:00 – 2:00 p.m. PT
Abstract Number: 1389

Title: Comparison of Cardiac Specific Promoters to Liver-specific miRNA Targets to Maximize Cardiac vs Liver Expression Following Intravenous AAVrh.10-mediated Cardiac Gene Therapy
Presenter: Abhishek Bose, Ph.D., Weill Cornell Medicine
Date/Time: Friday, May 19, 12:00 – 2:00 p.m. PT
Abstract Number: 1518

Title: Safety of Ascending Doses of AAVrh.10hFXN to Treat the Cardiac Manifestations of Friedreich's Ataxia
Presenter: Jonathan B. Rosenberg, Ph.D., Weill Cornell Medicine
Date/Time: Friday, May 19, 12:00 – 2:00 p.m. PT
Abstract Number: 1656

CNS Posters

Title: Modification of the AAV Serotype rh.10 Capsid to Enhance CNS Gene Transfer
Presenter: Kalpita R. Karan, Ph.D., Weill Cornell Medicine
Date/Time: Thursday, May 18, 12:00 – 2:00 p.m. PT
Session Title: Thursday Poster Session
Abstract Number: 835

Title: Suppression of Neurological Deterioration in the APP1.PSEN1/APOE4 Murine Model of Alzheimer’s Disease by AAV-mediated Expression of APOE2 Christchurch
Presenter: Caner Günaydin, Ph.D., Weill Cornell Medicine
Date/Time: Thursday, May 18, 12:00 – 2:00 p.m. PT
Abstract Number: 816

All abstracts for the ASGCT Annual Meeting are available on ASGCT's website.

**Is a paid consultant and has equity ownership in LEXEO Therapeutics, Inc.*

About LEXEO Therapeutics
LEXEO Therapeutics is a New York City-based, clinical-stage gene therapy company focused on addressing some of the most devastating genetically defined cardiovascular and central nervous system diseases affecting both larger-rare and prevalent patient populations. LEXEO's foundational science stems from partnerships and exclusive licenses with leading academic laboratories at Weill Cornell Medicine and the University of California, San Diego. LEXEO is advancing a deep and diverse pipeline of AAV-based gene therapy candidates in rare cardiovascular diseases and APOE4-associated Alzheimer's disease, and is led by pioneers and experts with decades of collective experience in genetic medicines, rare disease drug development, manufacturing, and commercialization. For more information, please visit www.lexeotx.com or LinkedIn.

Media Response:
Janine Bogris
(201) 245-6838
janine.bogris@canalecomm.com