LEXEO Therapeutics Expands Cardiac Gene Therapy Pipeline with Acquisition of Stelios Therapeutics and its Gene Therapy Programs for Rare Cardiovascular Diseases

LEXEO obtains exclusive rights to three investigational AAV-mediated gene therapy programs for rare cardiac disorders, all of which have no existing disease-modifying treatments available

Strategic acquisition expands LEXEO's rare cardiac gene therapy pipeline making it one of the most extensive in the field

NEW YORK – July 21, 2021 (GLOBE NEWSWIRE) – LEXEO Therapeutics, a fully integrated clinical-stage gene therapy company advancing disease-modifying treatments for genetic conditions, today announced that it has acquired Stelios Therapeutics, an early-stage company developing novel adeno-associated virus (AAV)-mediated gene therapies for rare genetic cardiac conditions. Through the agreement, LEXEO obtains exclusive rights to three preclinical AAV-mediated gene therapy programs focused on TNNI3-associated hypertrophic cardiomyopathy (HCM) and arrhythmogenic right ventricular cardiomyopathy (ARVC), two rare cardiac disorders with no approved pharmacological treatments and significant commercial potential. Stelios' Scientific Co-founder and Professor of Medicine in Cardiology at the University of California San Diego School of Medicine (UC San Diego), Dr. Eric Adler, will assist in cardiovascular research efforts at LEXEO Therapeutics.

The acquisition allows LEXEO to expand its pipeline in rare genetic cardiac diseases with the addition of three novel programs. The transaction provides a unique opportunity to integrate LEXEO and Stelios' complementary capabilities, including LEXEO's growing infrastructure in clinical development and chemistry, manufacturing and controls (CMC) with an existing focus on rare cardiac diseases, and Stelios' robust pre-clinical pipeline and domain expertise in rare genetic cardiac diseases. The Stelios programs complement and broaden LEXEO's advanced pre-clinical gene therapy pipeline in rare cardiac diseases, which currently includes LX2006, an IV-administered, AAV-mediated gene therapy program for the potential treatment of cardiomyopathy associated with Friedreich's ataxia (FA).

"While we are in the early stages of applying the potential of AAV gene therapy to rare cardiac diseases, the possible therapeutic benefits across a broad number of previously untreated and currently underdiagnosed cardiac diseases are immense," said R. Nolan Townsend, Chief Executive Officer of LEXEO Therapeutics. "LEXEO and Stelios share the same commitment to advancing therapies for patients with rare cardiac diseases. The combined company will establish a leading position in the field of AAV cardiac gene therapy, enabled by a strong scientific footprint and a significantly enhanced pipeline, with the potential to move multiple programs into the clinic in the coming years to address substantial patients' needs in various indications."

Dr. Adler commented, "Despite recent medical advancements in the rare cardiovascular field, many rare cardiac diseases remain underdiagnosed and undertreated. There is an urgent need for disease-modifying solutions to address conditions such as TNNI3-associated HCM."

Dr. Farah Sheikh, Co-founder of Stelios Therapeutics and Professor of Medicine in Cardiology at the University of California San Diego School of Medicine, with a primary research focus on

translational efforts with ARVC, commented, “There are limited efforts in addressing the needs of ARVC patients. We are committed to making these patients a priority and excited to bring a therapy to the forefront.”

The transaction was approved by the Board of Directors of both companies and closed immediately. Financial terms of the transaction are undisclosed.

About TNNI3-associated Hypertrophic Cardiomyopathy (HCM) and Arrhythmogenic Right Ventricular Cardiomyopathy (ARVC)

HCM affects more than 650,000 people in the U.S. and is the second most common form of heart muscle disease, comprising 35-40% of cardiomyopathies. TNNI3-associated HCM patients represent approximately 5-7% of the HCM patient population, with an estimated disease prevalence of 30,000. The disease is caused by mutations in the TNNI3 gene encoding cardiac troponin I (cTnI), a protein that modulates cardiac muscle contraction and relaxation. It is associated with thickening and enlargement of the heart, leading to decreased blood flow and increased risk of heart failure and life-threatening arrhythmias. Current treatment options are limited to invasive surgical procedures and heart transplantation. There are no approved pharmacological treatments available. The commercial opportunity in TNNI3-associated HCM is estimated to surpass $2 billion in the U.S. and EU.

ARVC is a heart disease with genetic mutations that impair the structure and function of the heart muscle, resulting in cardiac cell death, fibrosis, heart dysfunction, rhythm abnormalities, and sudden death in young people. The US prevalence of ARVC is estimated at 1:2,000-1:5,000 people. More than 40% of ARVC patients die within 10-11 years of diagnosis and there are no disease-modifying treatments approved. The commercial opportunity in ARVC is estimated to surpass $4 billion in the U.S. and EU.

Preclinical data from the TNNI3-associated HCM gene therapy program has shown compelling expression of the human TNNI3 gene in the heart in mouse models. In addition, preclinical data from two ARVC gene therapy programs have demonstrated delivery of the gene that codes for the connexin 43 protein (Cx43) and the gene that encodes for Plakophilin-2 (PKP2), is associated with fewer arrythmias and the potential to increase survival. LEXEO plans to further optimize these programs and prepare for investigational new drug (IND)-enabling studies.

About Stelios Therapeutics, Inc.

Stelios Therapeutics is an early-stage company developing novel AAV-based gene therapies for rare genetic cardiac conditions. The company is founded based on research conducted at the University of California San Diego (UCSD) by a distinguished team of researchers and scientists with extensive experience and expertise in gene therapy drug development.

About LEXEO Therapeutics, Inc.

LEXEO Therapeutics is a fully integrated clinical-stage gene therapy company advancing disease-modifying treatments for genetic cardiovascular conditions and genetic conditions of the

central nervous system (CNS). The company aims to apply cutting-edge science to target the underlying causes of both rare monogenic diseases and diseases affecting large patient populations. LEXEO's current pipeline consists of adeno-associated virus (AAV)-mediated gene therapies in rare cardiac diseases, CLN2 Batten disease, and APOE4-associated Alzheimer's disease. In addition, the company has more than 15 AAV-mediated gene therapy programs in research and development. LEXEO was founded based on well-established gene therapy research legacy at Weill Cornell Medicine's Department of Genetic Medicine by a team of pioneering scientists, clinicians, and business leaders with deep expertise in gene therapy drug development. The company is headquartered in New York City. For more information, please visit www.lexeotx.com or LinkedIn.

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