Medera's Sardocor Granted FDA Orphan Drug Designation for DMD-associated cardiomyopathy Gene Therapy

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Sardocor Corp., the clinical-stage gene therapy subsidiary of Medera Inc., with a broad pipeline of adeno-associated virus (AAV)-based treatments for cardiovascular diseases, announced that it has been granted Orphan Drug designation (ODD) from the U.S. Food and Drug Administration (FDA) for the company's experimental gene therapy treatment for cardiomyopathy secondary to Duchenne muscular dystrophy (DMD). With an Investigational New Drug (IND) approval, the clinical trial, Modulation of SERCA2a In the Cardiomyopathy of Duchenne Muscular Dystrophy (MUSIC-DMD), is currently cleared to enroll patients. MUSIC-DMD will be part of the first-ever program to use gene therapy to modify the pathophysiology of DMD cardiomyopathy, a major problem in late-stage DMD patients.

DMD is a rare, genetic disease, primarily affecting males and characterized by progressive degeneration and weakness of both skeletal and cardiac muscle. Data have shown that DMD cardiomyopathy is nearly universally present by the age of 18 and then progresses to heart failure and is now the main cause of death in this disease, surpassing respiratory failure.

Sardocor's investigational agent is an AAV1-based gene therapy designed to deliver the gene for sarcoplasmic reticulum calcium ATPase (SERCA2a) as a one-time infusion in the main coronary arteries of the heart. SERCA2a is the protein responsible for controlling intracellular calcium ions. In DMD cardiomyopathy, the heart muscle cells have elevated intracellular calcium and decreased SERCA2a, leading to toxicity from the elevated resting calcium and a reduction of the heart's ability to pump blood throughout the body.

MUSIC-DMD will enroll patients with DMD cardiomyopathy, age 18 years or older, using an open-label, dose-escalation trial design including an observational control cohort. While the initial primary goal is to assess short- and long-term safety following intra-coronary infusion of the gene therapy product, clinical efficacy will also be assessed with imaging modalities to assess the progressive fibro-fatty infiltration which is responsible for cardiac deterioration in the disease.

"Obtaining ODD status for MUSIC-DMD advances our efforts to expeditiously treat this devastating complication of Duchenne disease. The ODD designation is an important milestone for Sardocor supporting the development of effective treatments for cardiomyopathies

associated with Duchenne," said Ronald Li, PhD, CEO and Founder of Medera, the parent company of Sardocor.

"Within the intricate dance of life, hearts falter in Duchenne muscular dystrophy, where the delicate balance of calcium regulation in cardiomyocytes is disrupted. Delivery of SERCA2a via AAV-gene therapy has the potential to postpone the worsening of cardiomyopathy and extend the life of those living with Duchenne. We are very enthusiastic to see the continued development of this therapy" said Pat Furlong, President and CEO of Parent Project Muscular Dystrophy & a member of the Scientific Advisory Board at Sardocor.

"There is a great unmet need for therapies to delay or reverse the progressive cardiomyopathy associated with Duchenne. As patients afflicted with Duchenne live longer, cardiomyopathy has been more prevalent and this novel approach of targeting calcium cycling by gene therapy will hopefully give these patients an effective therapeutic strategy," said Roger J. Hajjar, MD, Scientific Co-Founder of Medera and Sardocor.

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Duchenne muscular dystrophy (DMD) is a rare, life-threatening, genetic, sex-linked disorder affecting all skeletal as well as cardiac muscles. It is the most common and severe form of muscular dystrophy among children. It is caused by deficiency of dystrophin, a protein that provides mechanical support to muscle fibers, thus preventing injury due to eccentric stresses on the muscle cell wall.

In the United States, an estimated 200,000 people are affected by the disease with a male birth incidence of about 1 in 3,800 to 6,300. The average age of diagnosis is 5 years with progressive muscle weakness, leading to limitations in ambulation in early childhood and loss of ambulation by 12 years of age, on average. Progressive weakening of the pulmonary muscles, kyphosis and scoliosis results in respiratory failure and previously led to death in the patients' second decade of life. Mitigation of pulmonary deterioration, through effective home ventilation, spinal stabilization surgery and the use of corticosteroids has substantially prolonged overall survival with many patients living into their third decade of life.

The cardiomyopathy caused by DMD presents later in life but is detectable by imaging in over 90% of patients by the age of 18 years. Progressive fibro-fatty infiltration of heart muscle commonly evolves into symptomatic heart failure by the second decade of life and has overtaken respiratory failure as the main cause of mortality in DMD.

There are no evidence-based effective therapies for DMD cardiomyopathy with treatment generally following guideline-directed medical therapy established for dilated heart failure, also known as heart failure with reduced ejection fraction, in adult patients. Therefore, to bridge the gap of substantial unmet medical need, development of new mechanism-based approaches with better targeting of the pathophysiology underlying DMD could result in an improvement in

quality of life and potentially prolonged survival.

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Founded in 2014, Medera is a clinical-stage company dedicated to next-generation cell and gene therapies for difficult-to-treat and incurable diseases. Medera's preclinical and clinical programs target a range of cardiac, vascular and muscular indications. Medera operates via its two subsidiaries:

1) Novoheart is responsible for Disease Modelling and Drug Discovery using its proprietary, award-winning Human mini-Heart® technology, which offers a range of bioengineered human heart constructs including the world's first human heart-in-a-jar as healthy and diseased human hearts for testing drug toxicity and efficacy. The unique Human mini-Heart® platform enables Novoheart to model human-specific diseases and discover therapeutic candidates all in the context of human cells and tissues, free from species-specific differences. Before privatization by Medera, Novoheart was dual-listed on the Toronto Stock Exchange and Frankfurt Stock Exchange.

2) Sardocor is dedicated to the clinical development of novel therapies. Building upon the discovery platform of Novoheart, Sardocor aspires to create the shortest regulatory path to clinic for advancing next-generation cell and gene therapies, and has developed a therapeutic pipeline for a range of cardiac, vascular and muscular diseases including heart failure with preserved ejection fraction (HFpEF), Duchenne muscular dystrophy (DMD) cardiomyopathy, and pulmonary hypertension. With open INDs, our first-in-human gene therapy trials for HFpEF and DMD cardiomyopathy are ongoing and recruiting patients.

For more information, please visit:
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