MEDERA'S SARDOCOR ANNOUNCES FDA CLEARANCE OF IND FOR FIRST-IN-HUMAN GENE THERAPY CLINICAL TRIAL FOR DMD CARDIOMYOPATHY

BOSTON, MASSACHUSETTS, UNITED STATES, December 1, 2022 /EINPresswire.com/ --

• With modern therapeutic strategies to prolong life expectancy, an increasing number of Duchenne Muscular Dystrophy (DMD) patients survive to develop cardiomyopathy and heart failure, resulting in a near-universal reduction in heart function by the age of 18.

• Cardiomyopathy secondary to DMD has become the primary cause of death in this rare disease, surpassing pulmonary compromise.

• Currently there are no specific treatments for DMD cardiomyopathy.

• With FDA clearance of the IND, Sardocor is initiating a Phase 1b clinical trial for SRD-001, an adeno-associated virus (AAV)-based technology, as first-in-human gene therapy for DMD-related cardiomyopathy.

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 today that the US Food & Drug Administration (FDA) has approved the Modulation of SERCA2A In the Cardiomyopathy of Duchenne Muscular Dystrophy (MUSIC-DMD2), a phase 1b clinical trial. MUSIC-DMD2 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. Research has 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.

The investigational agent, referred to as SRD-001, 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 pumping calcium ions from the cell's cytoplasm into the sarcoplasmic reticulum tubular network for storage which leads to muscle relaxation and filling of the ventricular pump chamber. Subsequent release of the stored calcium then triggers muscle contraction and the heart's pumping action. In DMD cardiomyopathy, the heart muscle cells have elevated intracellular calcium and do not make enough SERCA2a, leading to toxicity from the elevated resting calcium and a reduction of the heart's ability to pump blood throughout the body (heart failure). In experimental models of DMD, targeted overexpression of the SERCA2a to restore protein expression and activity significantly improved the structure and function of the heart and the skeletal muscles, thereby reducing morbidity and mortality.

MUSIC-DMD2 will enroll patients with DMD cardiomyopathy, age 18 years or older, using an open-label, dose-escalation trial design including an observational, comparator cohort. While the initial primary goal is to assess short and long-term safety following intra-coronary SRD-001 infusion, clinical efficacy will also be assessed with cutting-edge imaging modalities to assess the progressive fibro-fatty infiltration which is responsible for cardiac deterioration in the disease.

"With the increasing morbidity and mortality caused by heart failure in an increasingly mature DMD population, there is a great unmet need for therapies to delay or reverse the unrelenting progressive cardiomyopathy inherent to the ultimate devastating clinical outcome. Current therapies based upon generic, small molecule approaches to heart failure with reduced ejection fraction (HFrEF) are mostly unvalidated in DMD and do not target the basic DMD disease mechanisms. SRD-001 has the potential to improve the defects in calcium trafficking in DMD and improve the clinical outcome in these desperate patients," added Jonathan Plehn, MD, Chief Medical Officer of Sardocor.

"The heart has been a major focus of Parent Project Muscular Dystrophy (PPMD) for many years. As therapies are developed to extend skeletal muscle function, our question has been 'is the heart built to last?'. Medera/Sardocor's approach to deliver SRD-001 to improve calcium exchange in the heart has the potential to improve the quality and life span of DMD patients, which is MUSIC to the ears of every DMD family," added Pat Furlong, President of PPMD.

"This IND for DMD cardiomyopathy is yet another landmark achievement for Medera/Sardocor and adds to the first-in-human Heart Failure with Preserved Ejection Fraction (MUSIC-HFpEF1) gene therapy trial that we recently announced. At Medera/Sardocor, we have culminated several decades of world-class pioneering knowledge in calcium cycling since the very initial discoveries of their pathway proteins. Their mechanistic actions, physiologically and pathophysiologically, have been well documented in the medical literature by us and others. Now is an opportune time to translate such knowledge into tangible patient benefits, and we are extremely excited about doing so," said Roger J. Hajjar, MD, Scientific Co-Founder of Medera and Sardocor.

"Given the central importance of calcium cycling in multiple cardiovascular diseases, we are taking a holistic, mechanism-based approach to develop novel therapeutics for treating DMD, HFpEF and other indications," said Ronald Li, PhD, CEO and Founder of Medera.

About DMD

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 and wheelchair confinement in early childhood. Progressive weakening of the pulmonary muscles, kyphosis and scoliosis results in respiratory failure and previously led to death in the patients' twenties. 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 thirties or even forties.

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

There are not many effective therapies for DMD cardiomyopathy which rely on standard, generic approaches to HFrEF. 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.

About Medera, Novoheart and Sardocor

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, including HFpEF, DMD and Pulmonary Hypertension. Medera operates via its 2 subsidiaries:

(1) Novoheart is responsible for Disease Modelling & Drug Discovery using our proprietary, award-winning 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 toxicity and efficacy. This platform enables us to uniquely model human-specific diseases and discover therapeutic candidates all in the context of human cells and tissues, free from species-specific differences.

(2) Sardocor is dedicated to the clinical development of novel therapies. Building upon Novoheart's bioengineered human tissue-based assays for disease modelling and drug discovery, Sardocor aspires to create efficient development programs of next-generation cell & gene therapies with a high success rate.

For more information, please visit:
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