MEDERA'S SARDOCOR ANNOUNCES FAST TRACK DESIGNATION AND DOSING OF 3 PATIENTS IN FIRST-IN-HUMAN HFpEF GENE THERAPY TRIAL

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- Heart failure is a global pandemic with an estimated 64.3 million cases worldwide.
- Heart failure with preserved ejection fraction (HFpEF) accounts for half of heart failure cases but lacks disease-modifying therapeutics.
- Sardocor has been granted Fast Track Designation (FTD) by the FDA for SRD-001 treatment.
- Aligned with the FDA Modernization Act 2.0, in-vitro human-based data collected using bioengineered healthy and HFpEF mini-Hearts® (proprietary technology from Medera's Novoheart) were included in our IND and FTD regulatory applications.
- In the Phase 1/2A clinical trial for SRD-001, an adeno-associated virus (AAV)-mediated first-in-human gene therapy for HFpEF, Sardocor successfully injected the first 3 patients via our proprietary intracoronary delivery technique.

Sardocor Corp., a clinical-stage gene therapy company solely owned by Medera, with a broad pipeline of cardiovascular indications based on adeno-associated virus (AAV)-based treatments , announces the enrollment and dosing of the first three patients in the Modulation of SERCA2a In Cardiomyopathy (MUSIC)- Heart Failure with Preserved Ejection Fraction (HFpEF) clinical trial. Heart failure is a global pandemic with an estimated 64.3 million cases worldwide. Half of all heart failure patients are diagnosed with HFpEF, characterized by abnormal ventricular relaxation and elevated filling pressures. Currently, HFpEF has limited disease-modifying therapeutic options.

The U.S. Food and Drug Administration (FDA) has granted Fast Track Designation (FTD) to Sardocor's investigational gene therapy treatment for HFpEF. Fast Track is a process designed to facilitate the development and expedite the review of new drugs intended to treat serious conditions and address unmet medical needs. MUSIC-HFpEF is a first-in-human trial using gene therapy to modify the underlying pathophysiology of HFpEF.

SRD-001 is an AAV-based gene therapy that is directly delivered to cardiac ventricular muscle cells via a Sardocor's proprietary intracoronary infusion system for enhancing protein expression and transgene functional activity. Studies have demonstrated that this one-time, outpatient,

heart-specific delivery system, requires significantly lower viral titers (less than 1/100-fold per patient compared to other conventional means) for efficient transduction, and does not depend on AAV serotype or tropism, thereby reducing or eliminating potential side effects.

Novoheart, another solely owned subsidiary of Medera focused on bioengineered Human mini-Heart® technology for disease modeling and drug discovery, previously generated the first bioengineered HFpEF Human mini-Heart® models which reproduce key disease characteristics.* The human models identify downregulation of SERCA2a, a protein instrumental in pumping cytosolic calcium during ventricular relaxation, as the major cause of calcium-handling defects in HFpEF, and further demonstrate that AAV-mediated overexpression of SERCA2a, using SRD-001, improves such disease-defining relaxation defects.

The central importance of SERCA in cardiac physiology has been extensively validated in the medical literature. Indeed, SERCA-2a is commonly pursued as an ultimate effector for modifying Ca-handling-related cardiac effects via gene transfer or other means. While the efficacy of this approach has been demonstrated in numerous animal and human models, certain previous efforts and human trial had also failed due to overly low viral titers and poor transduction efficiency.

“Using Novoheart’s unique Human mini-Heart® platform, we are also able to titrate and optimize the dosages for human heart transduction using Sardocor’s clinical-grade AAV vector, SRD-001. In accordance with the FDA Modernization Act 2.0, these informative preclinical human-based data were included in Sardocor’s successful IND and FTD applications for FDA regulatory approval,” said Kevin Costa, Ph.D., CSO and co-founder of Novoheart, a solely owned subsidiary of Medera.

MUSIC-HFpEF, currently ongoing at Duke University Medical Center and University of Texas Southwestern Medical Center, enrolls patients with HFpEF confirmed by rigorous exercise hemodynamics and gas exchange in an open-label, dose-escalation, 52-week Phase 1b/2a clinical trial of SRD-001, administered via Sardocor’s one-time intracoronary infusion methodology. While the initial primary goal is to assess safety following intra-coronary SRD-001 infusion, clinical efficacy will also be assessed based on resting and exercise hemodynamics in subjects with HFpEF confirmed by baseline hemodynamics. The secondary endpoints will assess the effects of SRD-001 on exercise tolerance, lusitropic parameters, chamber sizes, quality of life, and biomarkers in subjects with HFpEF.

“We are delighted in reaching this milestone for the treatment of patients with HFpEF. Receiving Fast Track Designation from the FDA reinforces Sardocor’s belief in SRD-001 for the treatment of HFpEF. We are proud to be advancing this investigational gene therapy treatment to address a disease that affects tens of millions of patients. Sardocor is positioned to validate SRD-001 for the treatment of multiple cardiovascular indications,” said Ronald Li, Ph.D., CEO and Founder of Medera, parent company of Sardocor.

“This trial will rigorously examine whether SRD-001 has beneficial effects on hemodynamics in patients with HFpEF at rest and during exercise. It will also explore whether gene therapy as tested in this trial can have disease modifying effects”, said Marat Fudim, MD, MHS, Advanced Heart Failure Specialist at Duke University who is the site principal investigator.

“The enrollment of the first patients in this gene therapy trial will serve to validate the large body of work that has implicated deficiency of calcium cycling in HFpEF”, said Roger Hajjar, MD, co-founder of Medera and Sardocor.

*https://www.einpresswire.com/article/598164042/medera-s-novoheart-partners-with-astrazeneca-on-first-bioengineered-human-models-of-heart-failure

About HFpEF
Heart failure (HF) is a global pandemic with an estimated 64.3 million cases worldwide and an increasing trend in prevalence. The annual global economic burden of HF is estimated at over US$100 billion. Accounting for 50% or greater of the overall HF population, HFpEF is an age-related condition that has become far more prevalent in recent years. This is partly due to an increasing dissemination of knowledge about the syndrome leading to greater identification of the condition, but also due to changes in lifestyle leading to deleterious metabolic effects on the cardiac myocytes. Individuals affected by HFpEF experience similar degrees of morbidity and mortality to that of patients with HF with reduced ejection fraction (HFrEF). Despite the growing epidemic of this emerging syndrome, HFpEF-focused interventional trials have had little success except for the use of sacubitril-valsartan (Entresto™) and the sodium glucose transporter-2 (SGLT-2) inhibitor empagliflozin (Jardiance™) for reducing cardiovascular mortality and heart failure hospitalization. However, these agents are not disease-modifying and there is a critical need for therapeutic interventions that target the abnormal physiological mechanisms involved in HFpEF.

About Medera 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. Medera operates via its two subsidiaries: 1) Novoheart is responsible for Disease Modelling and Drug Discovery using our 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 privatisation 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 cardiomyopathy, and pulmonary hypertension.

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