New gene therapy proves effective in treating severe heart failure

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Researchers at Mount Sinai School of Medicine have developed a new gene therapy that is safe and effective in reversing advanced heart failure. SERCA2a (produced as MYDICAR) is a gene therapy designed to stimulate production of an enzyme that enables the failing heart to pump more effectively. In a Phase II study, SERCA2a injection through a routine minimally invasive cardiac catheterization was safe and showed clinical benefit in treating this patient population and decreasing the severity of heart failure. The data were presented this week at the Heart Failure Congress of the European Society of Cardiology in Berlin.

"SERCA2a met the primary endpoints and appears to be safe and effective in people with advanced heart failure," said trial investigator Jill Kalman, MD, Associate Professor, Medicine, Cardiology, Director of the Cardiomyopathy Program, Mount Sinai School of Medicine. "There is a significant unmet need for treatments in this patient population, and these data indicate that SERCA2a is a promising option for them."

The CUPID (Calcium Up-regulation by Percutaneous administration of gene therapy In cardiac Disease) trial is a randomized, double-blind, placebo-controlled study, which enrolled 39 patients with advanced heart failure to study the safety and efficacy of SERCA2a. Patients were randomized to receive SERCA2a gene delivery in one of three doses or placebo and were evaluated over six months. The treatment is delivered directly to the patient's heart during a routine outpatient catheterization procedure.

Patients in the SERCA2a group demonstrated improvement or stabilization in symptoms, heart function, and severity of heart failure. They also saw an increase in time between cardiovascular events and a decrease in frequency of events. SERCA2a was found to be safe, with no increases in adverse events, disease-related events, laboratory abnormalities, or arrhythmias compared to placebo.

SERCA2a was developed by a team led by Roger J. Hajjar, MD, Research Director of Mount Sinai's Wiener Family Cardiovascular Research Laboratories and the Arthur & Janet Ross Professor of Cardiology, Medicine, and Gene and Cell Medicine, Mount Sinai School of Medicine. The team discovered the landmark potential of the treatment in 1999 and has been pursuing its potential as a gene therapy target in state-of-the-art specially built pre-clinical laboratories at Mount Sinai.

"Mount Sinai Heart is committed to developing ground-breaking therapies and bringing them from bench to bedside," said Valentin Fuster, MD, Director of Mount Sinai Heart, the Zena and Michael A. Wiener Cardiovascular Institute and the Marie-Josee and Henry R. Kravis Center for Cardiovascular Health, The Mount Sinai Medical Center. "We look forward to further study of this important treatment."

According to the U.S. Centers for Disease Control & Prevention, about 5.8 million Americans suffer from heart failure, and 670,000 new cases are diagnosed each year. One in five people who have heart failure die within one year of diagnosis. In 2010, heart failure will cost the United States $39.2 billion, including the cost of health care services, medications, and lost productivity. Heart failure is most often treated with aggressive medical and device therapy, but has no cure. The most common symptoms of heart failure are shortness of breath, feeling tired, and swelling in the ankles, feet, legs, and sometimes the abdomen.

Provided by The Mount Sinai Hospital