Gene therapy improved left ventricular and atrial function in heart failure by up to 25 percent

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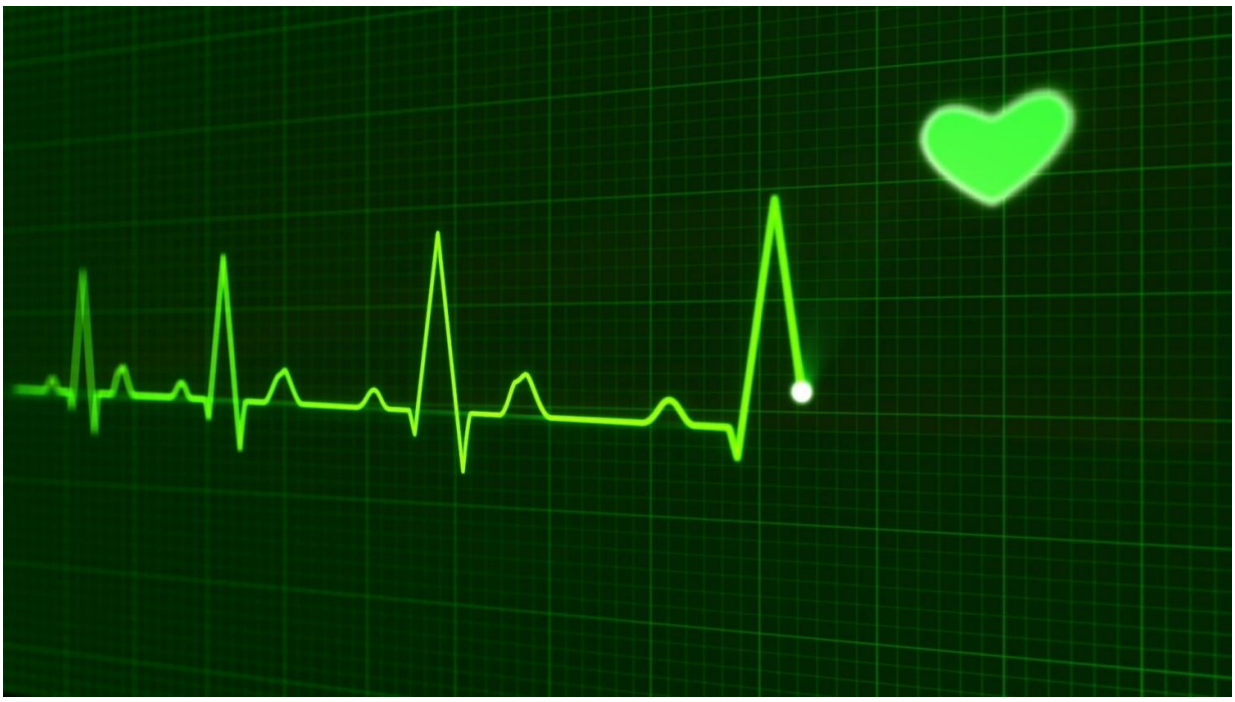

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Heart function improved by up to 25 percent in a trial using gene therapy to reverse cardiac damage from congestive heart failure in a large animal model, Mount Sinai researchers report. This is the first study using a novel vector for gene therapy to improve heart function in non-ischemic

heart failure.

The results of the study will be published online in the *Journal of the American College of Cardiology*.

In heart failure, a weakened or damaged heart no longer pumps blood effectively. This potentially fatal disease affects almost 6 million Americans, according to the American Heart Association, and is a major cause of morbidity and mortality, especially in elderly patients. Despite this toll, there has been little progress toward any kind of cure. Novel therapeutic approaches, such as gene therapy and cell therapy, hold the promise of complementing or replacing existing therapies for congestive heart failure.

"Mount Sinai has performed pioneering work on gene therapy over the last decade, and this study shows that gene therapy is now a viable option for treating congestive heart failure," said the study's senior author, Roger Hajjar, MD, Director of the Cardiovascular Research Center and the Arthur and Janet C. Ross Professor of Medicine at the Icahn School of Medicine at Mount Sinai. "There is a critical need to explore new therapeutic avenues and approaches."

This study featured two independent experiments. The first established the safety of administering a therapeutic gene delivery vector, BNP116, created from an inactivated virus over three months, into 48 pigs without heart failure through the coronary arteries via catheterization using echocardiography. The second experiment examined the efficacy of the treatment in 13 pigs with severe heart failure induced by mitral regurgitation. Six pigs received the gene and 7 received a saline solution.

The researchers determined that the gene therapy was safe and significantly reversed heart failure by 25 percent in the left ventricle and by 20 percent in the left atrium. Heart failure often results in enlarged

hearts, and the team found a 10 percent reduction of heart size in the affected animals. Heart failure in the cohort of pigs treated with saline worsened.

The research team plans to study the same gene therapy in a human trial starting next year. The gene vector has been approved by the Food and Drug Administration for clinical treatment.

Provided by The Mount Sinai Hospital