

# First gene therapy for heart failure offered at NewYork-Presbyterian/Columbia

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## **NYC's only medical center offering promising therapy as part of CUPID clinical trial**

Could injecting a gene into a patient with severe heart failure reverse their disabling and life-threatening condition? Physician-scientists are setting out to answer that question in a first-ever clinical trial of gene therapy to treat severe heart failure.

NewYork-Presbyterian Hospital/Columbia University Medical Center is the only center in the New York City area where the therapy is currently available.

Patients enrolled in the multicenter CUPID trial (Calcium Up-Regulation by Percutaneous Administration of Gene Therapy in Cardiac Disease) will undergo a minimally invasive cardiac catheterization procedure that will introduce a specially engineered gene that stimulates production of an enzyme necessary for the heart to pump more efficiently.

"This new therapy seeks to replenish the levels of this enzyme by introducing the gene for SERCA2a, which is depressed in these patients. If proven effective, this approach could be an alternative to heart transplant for patients without any other options," says Dr. Donna Mancini, the study's principal investigator at NewYork-Presbyterian Hospital/Columbia University Medical Center, where she is medical director of cardiac transplantation. She also is professor of medicine at Columbia University College of Physicians and Surgeons.

Gene therapy is a technique for correcting defective genes responsible for disease development by inserting genes into a patient's cells and tissues. In most gene therapy studies, a "normal" gene is inserted into the genome to replace an "abnormal" disease-causing gene. A carrier molecule called a vector must be used to deliver the therapeutic gene to the patient's target cells. Currently, the most common vector is a non-pathogenic virus most people have been exposed to in adolescence that has been genetically altered to carry normal human DNA.

More than five million people in the U.S. have heart failure. Patients with severe form of the disease have trouble breathing because the heart cannot pump fluid out of their lungs. Seventy percent die of the disease within 10 years, and the five-year survival rate is less than 50 percent. Heart failure is the only cardiovascular disease whose incidence has been increasing rather than decreasing.

Source: New York- Presbyterian Hospital/Columbia University Medical Center

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