

Cardiac stem cell therapy may heal heart damage caused by Duchenne muscular dystrophy

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Researchers at the Cedars-Sinai Heart Institute have found that injections of cardiac stem cells might help reverse heart damage caused by Duchenne muscular dystrophy, potentially resulting in a longer life expectancy for patients with the chronic muscle-wasting disease.

The study results were presented today at a Breaking Basic Science presentation during the American Heart Association Scientific Sessions in Chicago. After laboratory mice with Duchenne [muscular dystrophy](#) were infused with cardiac [stem cells](#), the mice showed steady, marked improvement in [heart](#) function and increased exercise capacity.

Duchenne muscular dystrophy, which affects 1 in 3,600 boys, is a neuromuscular disease caused by a shortage of a protein called dystrophin, leading to progressive muscle weakness. Most Duchenne [patients](#) lose their ability to walk by age 12. Average life expectancy is about 25. The cause of death often is heart failure because the dystrophin deficiency leads to cardiomyopathy, a weakness of the [heart muscle](#) that makes the heart less able to pump blood and maintain a regular rhythm.

"Most research into treatments for Duchenne muscular dystrophy patients has focused on the skeletal muscle aspects of the disease, but more often than not, the cause of death has been the heart failure that affects Duchenne patients," said Eduardo Marbán, MD, PhD, director of

the Cedars-Sinai Heart Institute and study leader. "Currently, there is no treatment to address the loss of functional heart muscle in these patients."

During the past five years, the Cedars-Sinai Heart Institute has become a world leader in studying the use of stem cells to regenerate heart muscle in patients who have had heart attacks. In 2009, Marbán and his team completed the world's first procedure in which a patient's own heart tissue was used to grow specialized heart stem cells. The specialized cells were then injected back into the patient's heart in an effort to repair and regrow healthy muscle in a heart that had been injured by a [heart attack](#). Results, published in The Lancet in 2012, showed that one year after receiving the experimental stem cell treatment, [heart attack patients](#) demonstrated a significant reduction in the size of the scar left on the heart muscle.

Earlier this year, Heart Institute researchers began a new study, called ALLSTAR, in which heart attack patients are being infused with allogeneic stem cells, which are derived from donor-quality hearts.

Recently, the Heart Institute opened the nation's first Regenerative Medicine Clinic, designed to match heart and vascular disease patients with appropriate stem cell clinical trials being conducted at Cedars-Sinai and other institutions.

"We are committed to thoroughly investigating whether stem cells could repair heart damage caused by Duchenne muscular dystrophy," Marbán said.

In the study, 78 lab mice were injected with cardiac stem cells. Over the next three months, the lab mice demonstrated improved pumping ability and exercise capacity in addition to a reduction in heart inflammation. The researchers also discovered that the stem cells work indirectly, by

secreting tiny fat droplets called exosomes. The exosomes, when purified and administered alone, reproduce the key benefits of the [cardiac stem cells](#).

Marbán said the procedure could be ready for testing in human clinical studies as soon as next year. The process to grow cardiac-derived stem cells was developed by Marbán when he was on the faculty of Johns Hopkins University. Johns Hopkins has filed for a patent on that intellectual property and has licensed it to Capricor, a company in which Cedars-Sinai and Marbán have a financial interest. Capricor is providing funds for the ALLSTAR clinical trial at Cedars-Sinai.

The Cedars-Sinai Heart Institute has been at the forefront of developing investigational stem cell treatments for heart attack patients.

Provided by Cedars-Sinai Medical Center

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