Heart drug improves or stabilizes heart function in Duchenne muscular dystrophy

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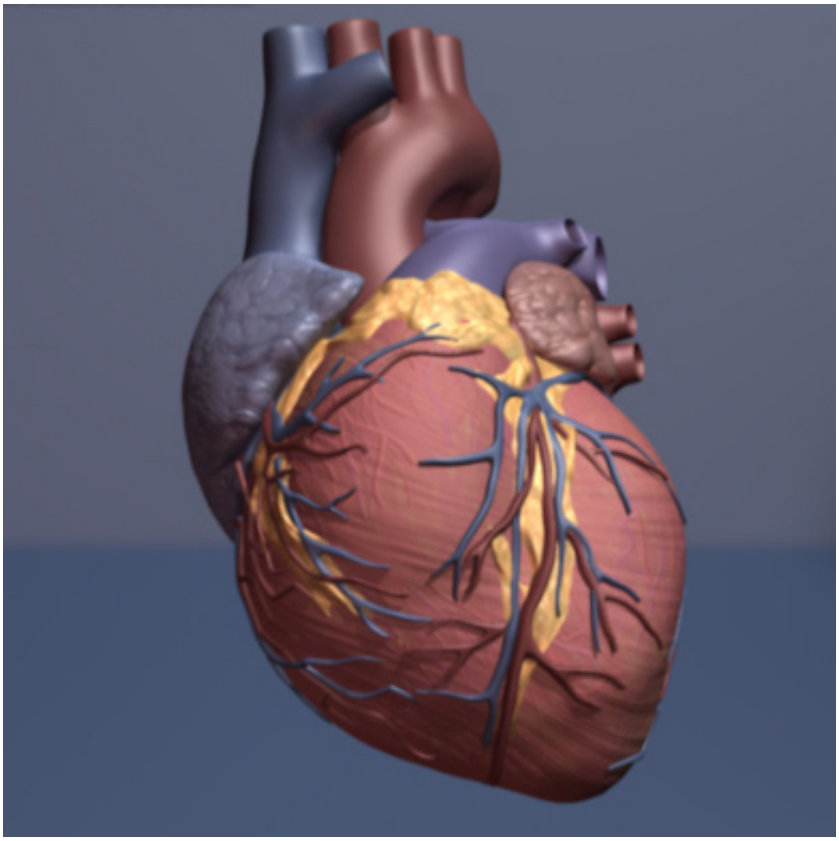

Human heart. Credit: copyright American Heart Association

Researchers at The Ohio State University Ross Heart Hospital and Nationwide Children's Hospital have shown early treatment with the heart failure medication eplerenone can improve heart function in young boys with Duchenne muscular dystrophy (DMD) and stabilize heart

function in older boys with the disease.

The results of their study are published in *Orphanet Journal of Rare Diseases*.

The scientists examined the safety and efficacy of longer-term therapy with eplerenone, a diuretic used to treat high blood pressure and heart failure. This directly followed their 12-month study of the drug, published in the *Lancet Neurology*, which showed it slowed the progressive decline in heart function among DMD patients, compared to placebo.

Eleven boys and young men, ages 7 to 25, from the original 12 month study went on to participate in this 24-month trial extension during which all participants received 25 mg of eplerenone daily to treat DMD heart disease.

Over two years, researchers examined a sensitive measurement of heart function known as strain. It starts becoming abnormal long before symptoms or other signs of heart disease appear.

"We saw significant improvement in left ventricular systolic function among the younger boys who were newly treated with eplerenone. The older patients who continued eplerenone therapy from the previous trial to this one remained stable," said Dr. Subha Raman, a cardiologist and professor at Ohio State Wexner Medical Center and lead author on the study. "Recognizing that cardiopulmonary failure remains the leading cause of death in this disease, this tells us we should strongly consider early use of this medication in boys with DMD in order to gain the greatest cardiac benefit."

Additionally, the research team reported no one experienced any adverse effects from the drug, such as elevated potassium levels.

DMD is a genetic disorder in which the body lacks dystrophin, a protein that helps keep muscle cells intact. It causes the skeletal and heart muscles to rapidly degenerate and weaken. DMD predominantly affects males. A majority of patients develop heart or respiratory failure, surviving into their 20s or 30s.

"It's important to remember that the heart is a muscle too. If we're to achieve long term improvements in duration and quality of life for patients with DMD, we must strive to understand the associated heart muscle disease better. This trial is one small piece of that big puzzle," said Dr. Linda Cripe, a pediatric cardiologist and co-investigator at Nationwide Children's.

This research was inspired by 30-year-old Ryan Ballou of Pittsburgh, a young man with DMD who, along with his father, started BallouSkies to raise awareness and funding for Raman's research of heart disease in muscular dystrophy patients. BallouSkies and the Parent Project for Muscular Dystrophy financially supported the study.

More information: Subha V. Raman et al. Eplerenone for early cardiomyopathy in Duchenne muscular dystrophy: results of a two-year open-label extension trial, *Orphanet Journal of Rare Diseases* (2017). DOI: 10.1186/s13023-017-0590-8

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