

Researchers discover chemical that may protect hearts of muscular dystrophy patients

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Researchers at the University of Minnesota Medical School have discovered a chemical that may, over the long term, protect the hearts of Duchenne muscular dystrophy patients - a fatal and most common form of muscular dystrophy in children.

The chemical, which Medical School scientists have termed a "molecular band-aid," seeks out tiny cuts in diseased heart muscle. When injected into the bloodstream, the molecular band-aid finds these microscopic cuts and protects them from harmful substances so the heart muscle cells can survive and function normally. In order to be effective the chemical must be repeatedly injected, much in the same way a diabetic patient requires regular injections of insulin,

In the March 15 edition of the <u>Journal of Clinical Investigation</u>, Joseph Metzger, Ph.D., professor and chair of the Department of Integrative Biology and Physiology, DeWayne Townsend, D.V.M., Ph.D., assistant professor in the Department of Integrative Biology and Physiology, and colleagues showed the first ever effective long-term treatment for preventing cardiac injury and progressive heart chamber remodeling in a severely affected canine model of muscular dystrophy.

In the study, dystrophic dogs were given the molecular band-aid continuously for two months. The treatment completely blocked cardiac injury and heart disease remodeling compared to the control group of dystrophic canines receiving a placebo.



"The advance in this study is demonstrating that molecular band-aid therapy is a safe and effective approach in preventing <u>heart damage</u> in severely affected large animals with muscular dystrophy," Metzger said.

The hopeful next major step is to determine whether children with muscular dystrophy can be helped by applying the molecular band-aid, first over short periods, then if successful, over the long term with the ultimate goal of enhancing the health and quality of life of muscular dystrophy patients.

Muscular dystrophy causes the muscles in the body to progressively weaken. Duchenne is the most common and severe form of childhood muscular dystrophy. About one of 3,500 boys are born with the crippling disease. Symptoms usually begin in children who are 4-5 years-old, most are in a wheelchair by age 12, and many who have the disease pass away by their late teens to early 20s. The primary causes of death are respiratory failure and heart failure. Current treatments, largely limited to corticosteroids, are minimally effective and can cause serious side effects.

The potential for the molecular band-aid discovery is yet to be fully realized - and may be stretched even beyond those who are impacted by muscular dystrophy. Metzger and Townsend believe the molecular bandaid may be applicable in elderly patients who simply have weakened heart muscle. If that is the case, the molecular Band-Aid could be used as a therapy for millions.

"We speculate that certain types of heart damage that occur when we age or when the heart is failing may also someday benefit from molecular band-aid therapy," Townsend said.

Provided by University of Minnesota



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