

While focusing on heart disease, researchers discover new tactic against fatal muscular dystrophy

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Based on a striking similarity between heart disease and Duchenne muscular dystrophy, researchers at Columbia University Medical Center have discovered that a new class of experimental drugs for heart failure may also help treat the fatal muscular disorder.

At first glance, heart failure and the muscle-wasting Duchenne disease couldn't appear more dissimilar. Duchenne affects boys usually before the age of 6, destroying their muscle cells. The boys become progressively weaker through their teens and usually die in their twenties. In people without Duchenne, heart failure typically starts much later in life, robbing the heart's pumping ability in the 7th, 8th or 9th decade of life.

But the new study found that the muscle cells affected in both diseases have sprung the same microscopic leak that ultimately weakens skeletal muscle in Duchenne and cardiac muscle in heart failure. The leak lets calcium slowly seep into the skeletal muscle cells, which are damaged from the excess calcium in Duchenne. In people with chronic heart failure, a similar calcium leak continuously weakens the force produced by the heart and also turns on a protein-digesting enzyme that damages its muscle fibers.

Andrew Marks, M.D., the study's leader, hypothesized that a new class of experimental drugs developed at CUMC - which he had designed to

plug the leak in the heart - could also work for Duchenne.

The drugs, when given to mice with Duchenne, dramatically improved muscle strength and reduced the number of damaged muscle cells.

"This was extremely exciting to us," says Dr. Marks, chair of the Department of Physiology & Cellular Biophysics and Clyde and Helen Wu Professor of Molecular Cardiology. "If it works in people, our drug won't be a cure, but it could slow the pace of muscle degeneration and extend the lives of people with Duchenne."

The study was published online Feb. 8 in *Nature Medicine*. Though the new drugs are not FDA-approved or currently available for Duchenne patients, a similar drug that was used in the Duchenne study is undergoing Phase I safety trials, and later this year trials will begin for heart failure.

Source: Columbia University Medical Center

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