

First phase II trial of heart disease treatment for Duchenne muscular dystrophy launched

EurekAlert

Baltimore, Md. -- Kennedy Krieger Institute announced today the launch of a first-of-its-kind, phase II clinical trial to investigate a treatment for heart disease in individuals with Duchenne muscular dystrophy (DMD). Made possible by a \$545,000 grant from Pilot Trials Now, an innovative DMD initiative organized and managed by Charley's Fund Inc. and The Nash Avery Foundation, with additional support from Pfizer Inc., the study will examine whether Revatio (sildenafil) improves cardiac function for those affected by the most common fatal genetic disorder in the world.

DMD affects approximately 1 in 3,500 boys worldwide. Errors in the gene that codes for dystrophin, a protein that plays a key role in muscle fiber function, result in progressive muscle-wasting that eventually spreads throughout the body, affecting both the heart and lung muscles, ultimately resulting in paralysis and death by early adulthood. With no cure for DMD, researchers hope that preserving the function of key muscles such as the heart and lungs can contribute to increased life expectancy.

The Kennedy Krieger Institute research team, working collaboratively with the Division of Cardiology in the Johns Hopkins University School of Medicine, will investigate whether Revatio (sildenafil), the same drug in Viagra which is best known for its efficacy in treating male erectile dysfunction, can improve cardiac function by preventing pathologic left ventricular hypertrophy, an unhealthy increase of the heart's muscle mass that outpaces the heart's pumping ability. In research first pioneered by co-investigator David Kass, Johns Hopkins' Abraham and Virginia Weiss Professor of Cardiology, sildenafil was shown to suppress and even reverse heart muscle dysfunction and hypertrophy in experimental animal models. Recently, research supported by Charley's Fund Inc, The Nash Avery Foundation and the Parent Project Muscular Dystrophy revealed cardiac benefits of sildenafil in mouse models of muscular dystrophy.

"We are very excited to be offering a clinical trial to this older Duchenne muscular dystrophy population with this promising drug," said Dr. Kathryn Wagner, Director of the Center for Genetic Muscle Disorders at Kennedy Krieger. "For the first time in decades, there are promising therapies in development that I believe should give families a real sense of hope for the future."

Thirty males, aged 15 years and older, who have been diagnosed with DMD and cardiac dysfunction, are being recruited to participate in this randomized, double-blind, placebo-controlled study for six months. An open-label period during which all enrolled subjects will receive Revatio (sildenafil) will follow for an additional six months. Dr. Daniel Judge, Medical Director for the Johns Hopkins Center for Inherited Heart Diseases, will co-direct the trial.

[SOURCE](#) [1]

Source URL (retrieved on 11/29/2014 - 12:41am):

<http://www.mdtmag.com/news/2010/10/first-phase-ii-trial-heart-disease-treatment-duchenne-muscular-dystrophy-launched>

Links:

[1] http://www.eurekalert.org/pub_releases/2010-10/kki-fpi101110.php