MedicalXpress

Successful regeneration of human skeletal muscle in mice enables accelerated research in muscular dystrophy

January 27 2014

Researchers at the Kennedy Krieger Institute recently announced study findings showing the successful development of a humanized preclinical model for facioscapulohumeral muscular dystrophy (FSHD), providing scientists with a much needed tool to accelerate novel therapeutic research and development.

Published in *Human Molecular Genetics*, the study outlines the validity of a unique model that, for the first time, mirrors the gene expression and biomarker profile of human FSHD tissue. Previously, there has been no accepted preclinical model for FSHD, a complex and rare neuromuscular disorder that affects approximately 4-7 per 100,000 individuals. As a result, therapeutic development for the disorder has been stymied.

"The inability to mimic the FSHD's genetic mechanism in preclinical models has been an ongoing challenge for the research community. Without an accurate model, making the leap to clinical research commonly fails," said Kathryn Wagner MD, PhD, director of the Center for Genetic Muscle Disorders at the Kennedy Krieger Institute in Baltimore, MD. "We believe this unique model will open the door to studying muscle regeneration over time and help better predict clinical response to therapeutic drugs."

Inspired by cancer preclinical models developed with human tumor

tissue, Dr. Wagner and her research team leveraged both basic science and clinical research resources available at Kennedy Krieger to successfully regenerate grafted muscle within the models. Human bicep muscle biopsies transplanted into models survived for over 41 weeks and retained features of normal and diseased tissue.

"This model is not only applicable to genetic muscle diseases for which we lack appropriate research models, but for other acquired muscle conditions," said Wagner. "Now there will be more research possibilities related to the overall impact of age and disease on the regenerative and growth capacity of human skeletal muscle."

Provided by Kennedy Krieger Institute

Citation: Successful regeneration of human skeletal muscle in mice enables accelerated research in muscular dystrophy (2014, January 27) retrieved 6 May 2024 from https://medicalxpress.com/news/2014-01-successful-regeneration-human-skeletal-muscle.html