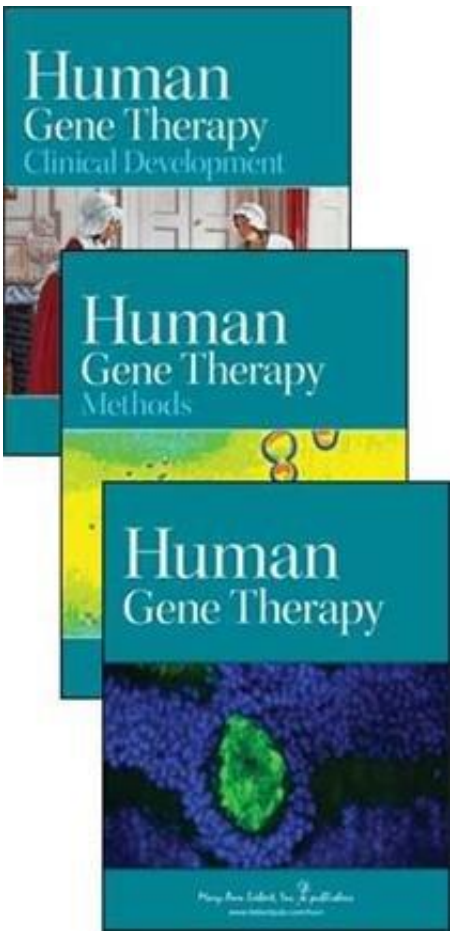
Alternative strategy for gene replacement shows promise in Duchenne muscular dystrophy

September 29 2015



Credit: Mary Ann Liebert, Inc., publishers

A gene therapy approach to treating the progressive muscle wasting

disorder Duchenne muscular dystrophy (DMD) that does not replace the mutated DMD gene but instead delivers the gene for ITGA7, a protein in skeletal muscle, led to reduced symptoms and significantly extended life span in a mouse model of severe DMD. Over-expression of ITGA7 did not elicit an immune reaction, further supporting its potential as a novel treatment for DMD, according to a new study published in *Human Gene Therapy*.

Kristin Heller, Louis Rodino-Klapac, and coauthors from The Research Institute at Nationwide Children's Hospital and The Ohio State University, Columbus, describe the method they used to deliver the α7 gene, which codes for the ITGA7 laminin receptor, to 5-7 day old mice deficient in both dystrophin and utrophin. These mice would usually die between 6-20 weeks of age, but gene transfer of α7 extended longevity by more than 10 weeks, as described in the article "Human α7 Integrin Gene (ITGA7) Delivered by Adeno-Associated Virus Extends Survival of Severely Affected Dystrophin/Utrophin-Deficient Mice".

"Recombinant AAV vectors are showing signs of clinical efficacy in a number of genetic disorders," says Editor-in-Chief Terence R. Flotte, MD, Celia and Isaac Haidak Professor of Medical Education and Dean, Provost, and Executive Deputy Chancellor, University of Massachusetts Medical School, Worcester, MA. "Certain genetic and immunologic aspects of Duchenne muscular dystrophy have made it a very complex target for simple rAAV-mediated gene replacement. This alternative strategy holds great promise for circumventing those potential issues to achieve clinical benefit in these patients."

More information: The article is available free on the *Human Gene Therapy* website until October 29, 2015.