# CRISPR-based gene therapy may provide cure to Duchenne muscular dystrophy

**The GLP aggregated and excerpted this blog/article to reflect the diversity of news, opinion and analysis.**

In an amazing, salutary coincidence, three independent research groups recently reported progress utilizing gene modification approaches to reducing the muscular dysfunction characteristic of sex-linked Duchenne-type Muscular Dystrophy, the most common and severe form of the disease.

The current studies were done on mice, but in this instance mouse and human gene mutations are quite similar, both biochemically and pathophysiologically re: the muscular dysfunction resulting from the genetic aberration.

Duchenne muscular dystrophy (DMD) is the most common fatal genetic disorder diagnosed in childhood, affecting approximately one in every 3,500 live male births (about 20,000 new cases each year worldwide). Because the Duchenne gene is found on the X-chromosome, it primarily affects boys; however, it occurs across all races and cultures.

The new studies used a groundbreaking gene-altering/modification technique known as CRISPR/Cas-9 ( *clustered regularly interspaced short palindromic repeats*), which is analogous genetically to the “find and replace” function on document manipulation. The scientists attached dystrophin-specific CRISPR/Cas-9 technology to a virus (“AAV,” adeno-associated virus) and used the virus as a carrier to transport the corrective gene modifier to ailing muscle cells in mice.

**Read full, original post:** Progress on Gene Modification Rx for Muscular Dystrophy