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# CRISPR-Cas9 examined for potential and challenges treating Duchenne Muscular Dystrophy

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# CRISPR-Cas9 examined for potential and challenges treating Duchenne Muscular Dystrophy

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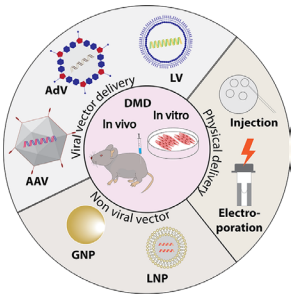
**Gene editing treatments are a promising solution to rare genetic diseases, but still face significant obstacles to implementation.**




Duchenne Muscular Dystrophy (DMD) is a lethal genetic disease found in one out of 5,000 male births. No treatments for DMD currently exist, but several gene therapy approaches are in various stages of clinical research.

Gene editing with CRISPR-Cas9 is a promising alternative approach that corrects the disease's underlying pathology, but major barriers prevent its application. Padmaswari et al. investigated CRISPR-Cas9 techniques and challenges treating DMD.

DMD is a recessive, X-chromosome-linked disorder caused by mutated dystrophin, a gene that helps stabilize muscle membranes. Symptoms manifest in 3- to 5-year-old patients as frequent falls and weakness. Muscle degradation progresses until cardiac or respiratory failure causes death by early adulthood.

The varied mutations that cause DMD require multiple approaches for treatment. CRISPR can make some genes functional by removing damaged parts, or a 'knock-in' method can replace missing functional components. Yet another technique alters a similar gene's behavior to perform dystrophin's functions.

Despite flexibility in treatment options, CRISPR-Cas9 gene editing faces significant challenges. Full-length dystrophin is too large for common delivery vectors and some gene editors. Because the Cas9 enzyme is found in common bacteria, the treatment may be blocked or reversed by the immune system, reducing its efficiency. CRISPR techniques can suffer from a lack of precision in applying corrections. And the recent emergence of gene-editing means there is no long-term data on patient outcomes for CRISPR-Cas9 DMD treatments.

"CRISPR DMD treatment has come really far since its inception," said author Mary Jia. "There have been so many studies, and so many people have been investigating ways that we can fix this. We won't stop investigating until we find that curative therapy for patients."

**Source:** "Delivery challenges for CRISPR-Cas9 genome editing for duchenne muscular dystrophy," by Made Harumi Padmaswari, Shilpi Agrawal, Mary S. Jia, Allie Ivy, Daniel A. Maxenberger, Landon A. Burcham, and Christopher E. Nelson, *Biophysics Reviews* (2023). The article can be accessed at https://doi.org/10.1063/5.0131452.