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Gene Technology

Short Communication

CRISPR-Cas9: Advanced Management for Duchenne Muscular Dystrophy

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DESCRIPTION

Duchenne Muscular Dystrophy (DMD) is an inherited muscle disorder that affects approximately one in every 3,500-5,000 male births worldwide. It is a genetic mutation that results in muscle weakness and degeneration, leading to progressive disability. The disease is caused by a lack of the protein dystrophin, which helps keep muscles stable and functioning properly. Without it, muscle cells become weak and break down over time, leading to impairments in walking, running, jumping, climbing stairs and even sitting up [1]. As the disease progresses, other systems like the lungs and heart become affected as well. The impact of DMD can be devastating for those affected by the disorder and their families. Often families must cope with mounting medical bills due to frequent hospital stays or physical therapy visits. They may also need to make special arrangements for home care or modifications for wheelchairs or other mobility aids. The emotional toll of watching a child's mobility decline can be devastating for parents who want nothing more than to see their child reach their full potential [2]. Fortunately, recent advances in gene therapy have provided new hope for those affected by DMD. *CRISPR*-based gene editing technology has enabled scientists to identify mutations responsible for DMD and potentially create treatments that could replace defective genes with working copies of the dystrophin protein. This could potentially provide an effective treatment for DMD patients who are currently unable to access curative therapies due to financial or geographical barriers or whose disease has progressed too far for existing therapies to be effective [3].

CRISPR-based gene therapy has been gaining traction as a potential treatment for Duchenne Muscular Dystrophy (DMD) due to its ability to precisely control gene expression. DMD is a genetic disease that affects the muscles, causing progressive muscle weakness and loss of movement. It is typically caused by the mutation or absence of a protein called dystrophin, which helps maintain the integrity of muscle tissue [4]. *CRISPR* is an acronym that stands for Clustered Regularly Interspaced Short Palindromic Repeats and refers to a short sequence of Deoxyribo Nucleic Acid (DNA) within bacterial cells. The technology uses enzymes known as *Cas9* nucleases to modify sections of DNA in living cells. By targeting and correcting mutated genes responsible for DMD, it is thought that *CRISPR*-based gene therapy may be able to restore dystrophin production or slow down the progression of symptoms [5]. Although the technology is still in its early stages, there has been using *CRISPR*-based gene therapy for treating DMD. For example, one study found that when mice with DMD were given a single dose of gene therapy using *CRISPR*, they had reduced inflammation and improved muscle strength compared to untreated mice with DMD [6].

Similarly, another study found that injecting mice with *CRISPR*-based gene therapy improved their muscle function and reduced inflammation and scarring around their muscles. These studies suggest that *CRISPR*-based gene therapy could be an effective way to treat certain types of DMD in humans in the future [7].

The field of gene editing using *CRISPR-Cas* technology has brought renewed hope for individuals with Duchenne Muscular Dystrophy (DMD). *CRISPR-Cas* based gene therapy has been identified as one of the most potential treatments for DMD and has already shown capable results in animal models. In addition, clinical trials are currently underway to test its efficacy in humans [8]. This new form of gene therapy relies on harnessing the power of the *CRISPR* system to precisely edit genes that are responsible for producing dystrophin, a protein that is lacking in individuals with DMD. By editing the faulty genes, this form of therapy is capable of restoring normal levels of dystrophin and potentially eliminating the symptoms associated with DMD [9].

The potential benefits of this approach are numerous, including reducing physical disability and pain, improving quality of life, and ultimately leading to a cure for DMD. While there is much work still to be done before this form of gene therapy can become a reality, it marks an exciting breakthrough that could revolutionize the treatment and care of individuals afflicted with DMD. With continued clinical trials and it is possible that we may soon unlock the potential of *CRISPR*-based gene therapy for Duchenne Muscular Dystrophy and bring relief to those living with this debilitating condition [10].

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