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Poster Presentation

Adeno-Associated Viral Vectors in Duchenne Muscular Dystrophy

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Abstract

Duchenne muscular dystrophy (BMD) is an inherited X-link disease. The incidence of this muscle-wasting disease is 1:5000 male live births. Mutation in the gene coding for dystrophin is the main cause of BMD. Most cases of this disease succumb to respiratory and cardiac failure in 3rd to 4th decades. The slow progression of BMD and recent achievement of gene therapies make it as an appropriate candidate for this strategy to restore dystrophin production in most affected tissues. This review has focused on elucidating the role of Adeno-associated viral vectors in duchenne muscle dystrophy. Some strategies in gene therapy of BMD exon skipping, protein upregulation, stem cell transplants and mutation suppression in order to restore dystrophin production. Serious adverse events have been limited them. One of the novel and functional strategy to replace dystrophin is using shuttle vectors derived from adeno-associated virus (AAV). This method has been tested in numerous human clinical trials without life threatening adverse effects. Major limitations of AAV vectors include limited cloning capacity and activation of immune response. Therefore, using miniaturized dystrophin and effective methods in order to attenuate immune system can promote this strategy.

Keywords: Duchenne, Muscular Dystrophy, Adeno-Associated Viral (AAV) Vectors, Gene Therapy

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