

Hunting for a cure: the therapeutic potential of gene therapy in Duchenne muscular dystrophy

ABSTRACT

Duchenne muscular dystrophy (DMD) is an incurable disease and the search for a cure is a challenging journey. However, with recent encouraging progress, we are seeing a light at the end of a long tunnel. This review focuses on several main strategies in gene therapy, including truncated dystrophin gene transfer via viral vectors, antisense mediated exon skipping to restore the reading frame, and read-through of translation stop codons. An exon skipping agent, eteplirsen, and a termination codon read drug, ataluren, are currently the most promising therapies. With better understanding of the molecular mechanism, gene therapy has improved with regard to the key areas of gene stability, safety, and route of delivery. Consequently, it has emerged as an exciting and hopeful means for novel treatment of this devastating disease.

Keyword: Cure; Duchenne; Gene therapy; Muscular dystrophy