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# Pre- and posttranscriptional genetic information modification in muscular dystrophy treatment

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## Abstract

Nowadays, a whole range of genetherapeutic methods is being used to restore a lost protein function due to mutation, a big number of preclinical and clinical studies of potential drugs that may allow to implement an etiotropic approach is being performed. One of the most prevalent and socially significant groups of genetic pathologies is musculardystrophy, including such diseases as Duchenne muscular dystrophy and dysfelinopathy. Despite a large number ofstudies in this field, there is no effective method of gene therapy for these diseases yet. This work is intended to review main genetherapeutic methods in myodystrophy treatment, especially pre- and posttranscriptional genetic (biosynthetic) information modification, and analyze most optimal of them.

## Keywords

Clinical trials, Duchenne myodystrophy, Dysferlinopathy, Exon-skipping, Gene therapy, Muscular dystrophy, Trans-splicing