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Formation of the recombinant adenovirus encoding codon-optimized dysferlin gene and analysis of the recombinant protein expression in cell culture in vitro

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Abstract

Dysferlinopathies belong to neuromuscular diseases associated with aberrant expression and/or function of dysferlin protein in skeletal muscle, which is caused by mutations in the dysf (dystrophy-associated fer-1-like, DYSF) gene. Because of the large size of the codon-optimized dysf coding region (6243 bp), adenoviral vectors are suitable for the creation of genetic constructs, which are capable of delivering a large amount of recombinant genetic information into both dividing and non-dividing cells, as well as provide a high level of transgene expression. We generated a recombinant adenovirus serotype 5 encoding a codon-optimized gene for human dysferlin (Ad5-Dysf) and analysed recombinant protein expression in vitro in HEK-293T cell line.

Keywords

Codon optimization, Dysferlin, Gene therapy, Recombinant adenovirus