Review Curr Gene Ther. 2020;19(6):386-394. doi: 10.2174/1566523220666200218113526.

Limb-girdle Muscular Dystrophy and Therapy: Insights into Cell and Gene-based Approaches

Forough Taheri [1], Eskandar Taghizadeh [2] [3], Mohammad J R Pour [4], Daryoush Rostami [5], Pedram G Renani [1], Azam Rastgar-Moghadam [6], Seyed M G Hayat [7]

Affiliations

PMID: 32067617 DOI: 10.2174/1566523220666200218113526

Abstract

The Limb-Girdle Muscular Dystrophies (LGMD) are genetically heterogeneous disorders, responsible for muscle wasting and severe form of dystrophies. Despite the critical developments in the insight and information of pathomechanisms of limb-girdle muscular dystrophy, any definitive treatments do not exist, and current strategies are only based on the improvement of the signs of disorder and to enhance the life quality without resolving an underlying cause. There is a crucial relationship between pharmacological therapy and different consequences; therefore, other treatment strategies will be required. New approaches, such as gene replacement, gene transfer, exon skipping, siRNA knockdown, and anti-myostatin therapy, which can target specific cellular or molecular mechanism of LGMD, could be a promising avenue for the treatment. Recently, genome engineering strategies with a focus on molecular tools such as CRISPR-Cas9 are used to different types of neuromuscular disorders and show the highest potential for clinical translation of these therapies. Thus, recent advancements and challenges in the field will be reviewed in this paper.

Keywords: Adeno-associated virus; LGMD; exon skipping; gene editing; gene therapy; limb-girdle muscular dystrophy..

Related information

MedGen

LinkOut - more resources

Full Text Sources

Bentham Science Publishers Ltd.

Ingenta plc

Medical

Genetic Alliance

MedlinePlus Health Information