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Update on Muscular Dystrophies with Focus on Novel Treatments and Biomarkers.

Datta N¹, Ghosh PS².

Author information

Abstract

PURPOSE OF REVIEW: Muscular dystrophies are a heterogeneous group of inherited muscular disorders characterized by progressive muscle weakness and in many cases cardiac and respiratory muscle involvement. Historically, these disorders are considered incurable with grave prognoses. The genes responsible for most muscular dystrophies are known, and early diagnosis is achievable with proper clinical recognition and advanced genetic testing. This article reviews recent advances in the development of novel treatments and biomarkers in the realm of muscular dystrophies commonly encountered in pediatric population.

RECENT FINDINGS: The therapeutic landscape of muscular dystrophies has changed with the development of new approved treatments for Duchenne muscular dystrophy (DMD), the most common and severe muscular dystrophy. This has paved the way for the development of novel therapeutic strategies for not only DMD but also other muscular dystrophies. This article reviews recent advances in the development of novel treatments and biomarkers in the realm of muscular dystrophies commonly encountered in pediatric population.

KEYWORDS: Congenital muscular dystrophy; Duchenne muscular dystrophy; Emery-Dreifuss muscular dystrophy; Limb girdle muscular dystrophy; Muscular dystrophy; Myotonic dystrophy

PMID: 32409939 DOI: 10.1007/s11910-020-01034-6