



Pediatric Neurology Part III: Chapter 155. Innovating therapies for muscle diseases (Handbook of Clinical Neurology)

Annemieke Aartsma-Rus, Gert-Jan Van Ommen, Jean-Claude Kaplan

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The neuromuscular disorders (NMDs) involve many different genetic and acquired diseases. Corticosteroids (e.g., prednisone and deflazacort) are prescribed for some NMDs as a palliative treatment to slow down disease progression to some extent. For the vast majority of NMDs, no specific therapy is currently available that stops progression or reverses the clinical deficits of the diseases. However, recent progress with different therapeutic approaches is now resulting in numerous clinical trials. In this chapter, we give an overview of the current state of the art, opportunities and challenges for gene therapy, cell therapy, antisense-mediated modulation of splicing, and numerous drug therapies for NMDs in general, and Duchenne muscular dystrophy as a paradigm in particular. Although none of the proposed strategies has yet proven to be of therapeutic value in patients, it is reasonable to expect that clinical efficacy will soon be demonstrated for some of the more advanced approaches.

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