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Pediatric Neuromuscular Diseases: Current Concepts, Challenges and Opportunities

Guest Editor:

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Message from the Guest Editor

Pediatric neuromuscular disorders (NMD) in the era of precision medicine and disease-modifying treatment (DMT) have substantially different primary treatment strategies, courses, and outcomes now. Multidisciplinary team approaches, which are mandatory in the standards of care for paediatric NMD and long-term assessment, are required for facing the new natural history phenotypes, prolonged life expectancy, and disease course complications. The immunogenicity and immunological response to DMT, resistance to the second or the third line immunotherapy for chronic immune-mediated NMDs, and related pathogenic mechanisms open new research targets. New hopes are accompanied by new challenges for the scientific and clinical infrastructures supporting the development and delivery of DMT. The future is in the next generation of viral vectors, DMT with improved body-wide distribution, control elements limiting expression levels, the fine tuning of translation and protein expression, preventing neurodegeneration and even genotoxicity, including clinical trials enabling a cure for single patients with rare gene mutations, and new NMD treatment modalities and guidelines.

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