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Neuromuscular Disorders in Children and Adolescents

Guest Editor:

Prof. Rudolf Korinthenberg
Research Ethics Comittee, University of Freiburg, 79106 Freiburg, Germany

Deadline for manuscript submissions:
closed (10 December 2021)

Message from the Guest Editor

During the last decades, significant scientific and therapeutic progress has been achieved in neuromuscular disorders (NMD) of childhood. In 1985, Duchenne Muscular Dystrophy (DMD) was the first genetic disease whose genetic code could be deciphered. Since that time, continuously new genetic entities of NMDs were defined. Consequently, the classification of these diseases changed from a clinical-descriptive and formal-genetic to a molecular-genetic and pathophysiological one. This resulted in intensified research in the biology and therapy of these diseases, in the last years resulting in first effective gene-modifying treatments in DMD and Spinal Muscular Atrophy (SMA), and recently, gene replacement therapy in the most severe form of SMA. Despite this significant progress in therapeutic research, the way to cure or significantly improve life for most children and adolescents with neuromuscular disorders is still unknown and a long way off. Nevertheless, great progress has also been made in symptomatic and rehabilitative management, allowing one to significantly improve functioning and quality of life of the affected individuals and their families.

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Editor-in-Chief

Prof. Dr. Paul R. Carney
Departments of Child Health and Neurology, University of Missouri, 400 Keene Street, Columbia, MI 65201, USA

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Contact Us

Children Editorial Office
MDPI, St. Alban-Anlage 66
4052 Basel, Switzerland

Tel: +41 61 683 77 34
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children@mdpi.com