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Hereditary Neuromuscular Diseases

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Deadline for manuscript submissions:
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Message from the Guest Editors

Hereditary Neuromuscular diseases (NMDs) are a class of rare disorders affecting both paediatric and adult patients. Muscle function is affected in these patients as a consequence of direct or indirect pathologic mechanisms that can affect muscle fibers as well as motoneurons and the neuromuscular junction. In this issue, we want to provide an overview of NMDs ranging from the genetic cause to the characterization of natural history, disease pathogenesis, and new therapies. The characterization of NMDs has, in fact, recently moved to a new stage in which outcome measures have been developed and evaluated in order to provide a meaningful tool box to drug developers to test innovative drugs in interventional clinical trials. Ongoing new therapies including RNA modulation will be in focus. This issue will also cover new advances in preclinical therapeutic strategies ranging from the establishment of ex vivo proof of principle studies using muscle-derived stem cells to the delivery of specific drugs to skeletal muscles, and from gene editing to gene therapy.

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Editors-in-Chief

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