

## Special Issue

# Hereditary Neuromuscular Diseases

### 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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### Guest Editors

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### Deadline for manuscript submissions

closed (31 May 2019)



## Journal of Clinical Medicine

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There has been an explosion of gene and target based research and therapeutics in the multitude of fields that compose clinical medicine. The *Journal of Clinical Medicine's* (JCM) staff and editorial board are dedicated to providing cutting edge, timely, and peer-reviewed articles covering the diverse subspecialties of clinical medicine. The journal publishes concise, innovative, and exciting research articles as well as clinically significant articles and reviews that are pertinent to the myriad of disciplines within medicine. The articles published are relevant to both primary care physicians and specialists. The journal's full-texts are archived in PubMed Central and indexed in PubMed. Please consider submitting your manuscripts for publication to our journal and check us out on-line!

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