



Muscular Dystrophies: Pathophysiology and Therapy

Guest Editors:

Dr. France Piétri-Rouxel

Dr. Sestina Falcone

Prof. Dr. Edoardo Malfatti

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Message from the Guest Editors

This Special Issue, "Muscular Dystrophies: Pathophysiology and Therapy", will focus on the pathophysiological mechanisms underlying muscular dystrophies, new animal models and methodological approaches for studying muscle dysfunction/recovery, as well as the development of innovative therapeutic strategies and ongoing clinical trials.

Therapeutic strategies that address the root cause of these diseases are currently being developed for a handful of muscular dystrophies, including virus-based gene therapy and antisense drugs. In this context, the activation of the host immune system against the transgene or viral capsid, the route of administration and the accurately targeted tissue, in order to achieve the restoration of fully functional muscle in patients, remains a challenge. Therefore, strategies that aim to rescue altered proteins, as well as animal models and methodologies, must be optimized. These must be improved to achieve robust readouts, allowing the validation of treatment efficacy in clinical trials. This Special Issue is open to research studies, ranging from basic to preclinical approaches, and will also cover original articles and reviews.





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Editor-in-Chief

Prof. Dr. Felipe Fregni

1. Neuromodulation Center and
Center for Clinical Research
Learning, Spaulding
Rehabilitation Hospital and
Massachusetts General Hospital,
Harvard Medical School, Boston,
MA 02114, USA
2. Department of Epidemiology,
Harvard T.H. Chan School of
Public Health, Boston, MA 02115,
USA

Message from the Editor-in-Chief

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Biomedicines Editorial Office
MDPI, Grosspeteranlage 5
4052 Basel, Switzerland

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