

Special Issue

Unlocking the Future of Muscle Disease Treatment: Advancements in Gene Therapy, Antisense Therapeutics and Cutting-Edge Pharmacological Strategies

Message from the Guest Editors

Muscle disorders, including muscular dystrophy and various neuromuscular diseases, are remarkably heterogenic in terms of genetic defects, pathophysiology, and the age of onset. Unfortunately, effective treatments for these disorders have been elusive, principally due to their rarity and the necessity of often mutation-specific approaches and tailored therapies. However, in recent years, we have witnessed the emergence of novel therapies, such as gene therapy, antisense therapeutics, and pharmacological approaches. These breakthroughs have shown promise for the treatment of diseases like Duchenne muscular dystrophy and spinal muscular atrophy and ongoing advancements in the development of treatments for myotubular myopathy or limb girdle muscular dystrophy. These encouraging advancements demonstrate the feasibility of developing treatments for severe and complex muscle diseases using new tools and models and via collaboration between preclinical research laboratories and industries. Ultimately, this Special Issue seeks to shed light on the progress and potential of innovative therapeutic strategies for the treatment of muscular diseases.

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