Special Issue

Spinal Muscular Atrophy: Pathogenesis, Diagnosis, Therapeutics and Future Directions

Message from the Guest Editors

Spinal muscular atrophy (SMA) is a rare hereditary motor neuron disorder, with an estimated prevalence of 1 to 2 per 100,000 individuals. It is caused by the insufficient production of survival motor neuron (SMN) proteins due to homozygous deletion or mutation of the SMN1 gene. SMA is characterized by the degeneration of motor neurons in the spinal cord, leading to progressive muscle weakness and, in severe cases, respiratory failure.

In the past decade, groundbreaking therapeutic advances have dramatically altered the landscape of SMA treatment. New interventions, including SMN-targeted therapies such as gene therapy, antisense oligonucleotides, and SMN-enhancing drugs, have shown great potential in modifying the disease course and improving patient outcomes. These innovations mark a critical shift from symptomatic management to targeted therapies addressing the underlying genetic cause of SMA.The Editors of this Special Issue seek to gather high-quality papers that discuss these therapeutic breakthroughs, alongside emerging diagnostics and future directions in SMA management.

Guest Editors

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Message from the Editor-in-Chief

Biomedicines (ISSN 2227-9059) is an open access journal devoted to all aspects of research on human health and disease, the discovery and characterization of new therapeutic targets, therapeutic strategies, and research of naturally driven biomedicines, pharmaceuticals, and biopharmaceutical products. Topics include pathogenesis mechanisms of diseases, translational medical research, biomaterial in biomedical research, natural bioactive molecules, biologics, vaccines, gene therapies, cell-based therapies, targeted specific antibodies, recombinant therapeutic proteins, nanobiotechnology driven products, targeted therapy, bioimaging, biosensors, biomarkers, and biosimilars. The journal is open for publication of studies conducted at the basic science and preclinical research levels. We invite you to consider submitting your work to Biomedicines, be it original research, review articles, or developing Special Issues of current key topics.

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