Special Issue

Application of Biomarkers in Spinal Muscular Atrophy (SMA)

Message from the Guest Editor

Spinal muscular atrophy (SMA) is a rare, autosomal recessive, motor neuron neurodegenerative disease presenting wide phenotypic variability. SMA therapeutic landscape has been dramatically transformed with the introduction of three (nusinersen, onasemnogene abeparvovec, and risdiplam) disease-modifying therapies (DMTs) that increase the level of survival motor neuron (SMN) protein in the last decade. The identification and validation of biomarkers in SMA require sophisticated molecular techniques, including genomic sequencing, proteomics, and bioinformatics. Understanding the molecular pathways affected by SMA could lead to the discovery of new biomarkers. For example, research into the roles of neurotrophic factors, RNA splicing, and autophagy in SMA pathogenesis may uncover novel targets for therapy. This Special Issue will gather available evidence on the application of biomarkers in SMA. We invite researchers who are studying closely related disciplines to contribute original articles, reviews, and communications.

Guest Editor

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The International Journal of Molecular Sciences (*IJMS*, ISSN 1422-0067) is an open access journal, which was established in 2000. The journal aims to provide a forum for scholarly research on a range of topics, including biochemistry, molecular and cell biology, molecular biophysics, molecular medicine, and all aspects of molecular research in chemistry. *IJMS* publishes both original research and review articles, and regularly publishes special issues to highlight advances at the cutting edge of research. We invite you to read recent articles published in *IJMS* and consider publishing your next paper with us.

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