Special Issue

Individualised Therapies for Rare Disorders

Message from the Guest Editor

Rare diseases, though individually uncommon, collectively affect millions across the world and often present with severe, multisystem manifestations. Advances in molecular biology, genomics, and systems-level omics are now enabling a deeper understanding of pathogenic mechanisms and revealing actionable molecular targets. This Special Issue highlights cutting-edge research that drives individualised therapeutic strategies, from mechanistic discovery to translational application. We welcome studies that elucidate molecular pathways underlying rare disorders, identify biomarkers for the diagnosis or prediction of therapeutic response, and develop omics-guided stratification approaches. Contributions exploring innovative gene and RNA therapies, molecular-targeted agents, and personalised treatment paradigms—including n-of-1 or ultra-rare disease applications—are particularly encouraged. By integrating mechanistic insights with precision therapeutic design, this Special Issue aims to showcase how molecular science is reshaping the landscape of care for rare diseases and paving the way for safe, effective, and truly personalised interventions.

Guest Editor

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The *International Journal of Molecular Sciences* (*IJMS*) 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, and molecular biophysics. *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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