Special Issue

Rare Diseases: From Molecular Pathways to Therapeutic Strategies

Message from the Guest Editors

In the last two decades, rare diseases have covered an increasing interest in drug development research. The social demand to find solutions for unmet medical needs has boosted efforts to develop “orphan drugs”. Indeed, rare disorders, which are generally related to mutations in a specific gene, can be considered simplified models to study molecular pathways (i.e., autophagy blockage, inflammation, etc.) involved in broadly diffused pathologies, and therefore, they bring the opportunity to find transformative therapies with multiple therapeutic indications. On the other hand, rare diseases are also an ideal model to test recently developed chemical and biological tools for protein expression and genome editing, since they usually affect a single target protein. The scope of this issue is to describe the latest advances on rare diseases (i.e., metabolic diseases, neuromuscular disorders and other rare conditions with CNS involvement), facing both recent findings on physiopathological mechanisms and newly developed therapeutic approaches. This issue will welcome original research articles and up-to-date reviews on the described topics.

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Biomolecules is a multidisciplinary open-access journal that reports on all aspects of research related to biogenic substances, from small molecules to complex polymers. We invite manuscripts of high scientific quality that pertain to the diverse aspects relevant to organic molecules, irrespective of the biological question or methodology. We aim for a competent, fair peer review and rapid publication. Please look at some of the exciting work that has been published in *Biomolecules* so far. We would be delighted to welcome you as one of our authors.

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