

Special Issue

Rare Diseases: From Molecular Pathways to Therapeutic Strategies 2.0

Message from the Guest Editor

In recent decades, a massive improvement in biotechnological tools has paved the way to more efficient gene therapy strategies, which are currently under clinical trials or have successfully reached commercial authorisation. Gene therapy is a promising approach to cure many inborn errors of metabolism and neuromuscular disorders, as well as other rare disorders without a present effective treatment. The scientific community is more and more focused on the preclinical development of new possible gene therapy strategies, and the number of clinical trials for gene-therapy-based drugs has increased considerably with the aim of repeating the successful results of the recently approved drugs for Spinal Muscular Atrophy, Leber's Congenital Amaurosis, and Metachromatic leukodystrophy. The scope of this Special Issue is to describe the latest advances concerning new gene-therapy-based treatments, mainly focusing on metabolic diseases of genetic origin and neuromuscular disorders. This Special Issue will welcome original research articles and up-to-date reviews on the described topics. We look forward to reading your contributions.

Guest Editor

Dr. Saida Ortolano

Rare Diseases and Pediatric Medicine Research Group, Galicia Sur Health Research Institute (IIS Galicia Sur), SERGAS-UVIGO, Hospital Álvaro Cunqueiro, 36312 Vigo, Spain

Deadline for manuscript submissions

closed (30 September 2024)



Biomolecules

an Open Access Journal
by MDPI

Impact Factor 4.8
CiteScore 9.2
Indexed in PubMed



mdpi.com/si/141628

Biomolecules
Editorial Office
MDPI, Grosspeteranlage 5
4052 Basel, Switzerland
Tel: +41 61 683 77 34
biomolecules@mdpi.com

[mdpi.com/journal/
biomolecules](https://mdpi.com/journal/biomolecules)





Biomolecules

an Open Access Journal
by MDPI

Impact Factor 4.8
CiteScore 9.2
Indexed in PubMed



[mdpi.com/journal/
biomolecules](https://mdpi.com/journal/biomolecules)



About the Journal

Message from the Editorial Board

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.

Editors-in-Chief

Prof. Dr. Peter E. Nielsen

Department of Cellular and Molecular Medicine, Faculty of Health and Medical Sciences, University of Copenhagen, Blegdamsvej 3C, DK-2200 Copenhagen, Denmark

Prof. Dr. Lukasz Kurgan

Department of Computer Science, Virginia Commonwealth University, Richmond, VA 23284, USA

Author Benefits

Open Access

— free for readers, with article processing charges (APC) paid by authors or their institutions.

High Visibility:

indexed within Scopus, SCIE (Web of Science), PubMed, MEDLINE, PMC, Embase, CAPlus / SciFinder, and other databases.

Journal Rank:

JCR - Q1 (Biochemistry and Molecular Biology) / CiteScore - Q1 (Biochemistry)