

Special Issue

New Trends in Gaucher Disease: A Model for Rare Lysosomal Disorders

Message from the Guest Editors

Although rare world-wide (1:50,000–100,000), Gaucher Disease has a high prevalence among Ashkenazi Jews, and most of the patients with the so called "adult type" or type 1 live a normal lifespan, thereby allowing long term assessments as well as larger cohorts of patients compared to lethal disorders at a young age. There are also diverse animal models from different mice through drosophila fruit flies to zebra fish and human derived iPSCs, providing endless research opportunities. Gaucher disease was the very first lysosomal storage disease to have a safe and effective intravenous enzyme replacement therapy, to get market approval for oral substrate reduction therapy, and in addition, there are several additional treatment modalities such as pharmacological chaperones different gene therapy approaches. Still, there are many unmet needs and unresolved challenges, including the lack of treatment for the neuronopathic forms, the associations with common diseases such as various malignancies and neurodegenerative disorders, particularly Parkinson's disease. We look forward to leveraging the knowledge from Gaucher disease to the development of innovative therapies.

Guest Editors

Prof. Dr. Ari Zimran

Prof. Dr. Shoshana Revel-Vilk

Prof. Dr. Edward I. Ginns

Deadline for manuscript submissions

closed (30 September 2022)



International Journal of Molecular Sciences

an Open Access Journal
by MDPI

Impact Factor 4.9
CiteScore 9.0
Indexed in PubMed



mdpi.com/si/102329

*International Journal of
Molecular Sciences*
Editorial Office
MDPI, Grosspeteranlage 5
4052 Basel, Switzerland
Tel: +41 61 683 77 34
ijms@mdpi.com

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





International Journal of Molecular Sciences

an Open Access Journal
by MDPI

Impact Factor 4.9
CiteScore 9.0
Indexed in PubMed



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



About the Journal

Message from the Editor-in-Chief

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.

Editor-in-Chief

Prof. Dr. Maurizio Battino

Department of Odontostomatologic and Specialized Clinical Sciences,
Sez-Biochimica, Faculty of Medicine, Università Politecnica delle
Marche, Via Ranieri 65, 60100 Ancona, Italy

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, PMC, MEDLINE, Embase, CAPlus / SciFinder, and other databases.

Journal Rank:

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