Special Issue

Gaucher Disease: From Molecular Mechanisms to Treatments

Message from the Guest Editors

We are excited to invite you to be part of our new special issue entitled: “Gaucher Disease: From Molecular Mechanisms to Treatments”.

Gaucher disease is truly a remarkable model for rare diseases. It was among the first genetic disorders to demonstrate genotype–phenotype relationships using PCR-based methodology, and the first lysosomal storage disorder (LSD) to benefit from the orphan drug law three decades ago, and to have different therapeutic options.

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.

We are looking forward to receiving your contributions, and to what we believe might be an excellent up-to-date issue on all aspects of Gaucher disease, from basic science to clinical observations and therapies.

Guest Editors

Prof. Dr. Ari Zimran

Prof. Dr. Shoshana Revel-Vilk

Prof. Dr. Jeff Szer

Deadline for manuscript submissions

closed (30 October 2025)

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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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