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Lysosomal Storage Disorders: Molecular Basis and Therapeutic Approaches

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Message from the Guest Editor

Dear Colleagues,

After decades of efforts and limited advances, our knowledge on the biology of lysosomes has lately been dramatically increasing, thanks to the application of novel technologies. The lysosome no longer appears to be a simple "waste disposal bag", but rather a complex organelle with a plethora of important biological functions. Nonetheless, the growing body of knowledge on lysosomal storage disorders pathogenesis has led to a widespread awareness that alternative therapeutic approaches are required, beyond enzyme replacement therapy (ERT), in order to achieve the overall treatment of organ defects in affected patients.

In this Special Issue, we provide an updated state-of-the-art on lysosomal biology, with the integration of emerging concepts on lysosomal storage disorders' pathogenesis and novel challenging therapeutic perspectives and avenues.

Keywords

- Substrate storage
- Autophagy
- Cell signaling
- Biomarkers
- Gene therapy

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