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Lysosomal Storage Disease: From Molecular Mechanisms to Therapeutic Opportunities

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Deadline for manuscript submissions: closed (15 August 2021)

Message from the Guest Editor

Lysosomal storage disorders have been known for decades to be multisystem disorders with a variable onset and course. The molecular basis is increasingly being elucidated and therapeutic strategies are becoming more developed with growing expertise in targeted cellular and genetic therapies. Early diagnosis represents a challenge as therapeutic effects can mainly be achieved early in the disease course. Quite recent is interest in the role of lysosomes in a number of adult neurodegenerative disorders, such as Parkinson's disease and Alzheimer's disease. Although not of monogenetic origin, heterozygous and homozygous mutations in lysosomal genes are amongst the highest genetic risk factors and lysosomal dysfunction may enhance the neurodegenerative process.

We invite you to contribute to this Special Issue of Cells, which is dedicated to these disorders. Contributions on cell biology, molecular biology, and biophysics are as welcome, as are clinical studies covering the natural history or therapeutic aspects.









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