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Lysosomal Storage Disorders: Novel Concepts, Therapeutic Aspects and Beyond

Guest Editor:

Prof. Dr. Ritva Tikkanen
Institute of Biochemistry, Medical Faculty, Justus-Liebig University of Giessen, Friedrichstrasse 24, D-35392 Giessen, Germany

Deadline for manuscript submissions:
closed (28 February 2017)

Message from the Guest Editor

Dear Colleagues,

Lysosomal storage disorders (LSDs) are a heterogeneous group of rare monogenic diseases that are characterized by aberrant lysosomes with storage material. These diseases frequently manifest as severe defects of the central nervous system, mental retardation and reduced life span. Most LSDs result from a deficiency of a single enzyme, whereas others are caused by mutations in non-enzymatic proteins. In the past couple of years, our knowledge about the pathogenesis and the molecular details of the genes involved has substantially increased. These findings have forced us to rethink some old central dogmas about these diseases and revealed novel aspects about the pathomechanisms. Importantly, novel therapy options have become available, or are under development, for some LSDs that were previously considered fatal. In this Special Issue, We encourage the submission of review articles and original research papers of any length. For detailed information, you can refer to http://www.mdpi.com/journal/ijms/special_issues/lsd_nctab2016

Prof. Dr. Ritva Tikkanen
Guest Editor

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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

Message from the Editor-in-Chief

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International Journal of Molecular Sciences Editorial Office
MDPI, St. Alban-Anlage 66
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