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Management of Acute and Chronic Complications of Lysosomal Storage Diseases in Children and Adults: Current Practice and Future Opportunities

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Message from the Guest Editors

Dear Colleagues,

Lysosomal Storage Diseases manifest with clinical symptoms in childhood, although attenuated forms may present for the first time with subtle symptoms in adolescence or adulthood. The spectrum of clinical symptoms varies but the clear genotype–phenotype correlation is not well described for many of these conditions. Intrafamilial heterogeneity has been commonly observed but is not well understood.

Earlier diagnosis and advances in treatment have much improved the prognosis and life expectancy of patients with LSDs over the last decades. The increased survival has created a number of new issues and challenges: the development of long-term age-related complications, the metabolic progression of the underlying LSD, and the lack of data on the natural history of the disease. These new challenges require the care of adolescent LSD patients being transferred from metabolic paediatricians to metabolic physicians specialised in treating adults to an increasing extent, including the development and coordination of a multidisciplinary team for each individual LSD in tertiary centres.

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Editors-in-Chief

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