Special Issue

New Trends in Gaucher Disease: A Model for Rare Lysosomal Disorders

Message from the Guest Editors

Although rare world-wide (1:50,000–100,000), Gaucher Disease has a high prevenance among Ashkenazi Jews, and most of the patients with the so called "adult type" or type 1 live a normal lifespan, thereby allowing long term assessments as well as larger cohorts of patients compared to lethal disorders at a young age. There are also diverse animal models from different mice through drosophila fruit flies to zebra fish and human derived iPSCs, providing endless research opportunities. 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, including the lack of treatment for the neuronopathic forms, the associations with common diseases such as various malignancies and neurodegenerative disorders, particularly Parkinson's disease. We look forward to leveraging the knowledge from Gaucher disease to the development of innovative therapies.

Guest Editors

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