About Lysosomal storage diseases (**LSD**s)

Lysosomal storage diseases (LSDs) comprise a large group of rare inherited disorders, including Tay Sachs Disease, Fabry Disease, Gaucher's Disease, Pompe Disease, Hunter syndrome and other mucopolysaccharidosis (MPS). LSDs arise from enzyme deficiency resulting from inherited gene mutations. Specifically, the enzymes involved are required for metabolism of lipids or glycoproteins within cells; accumulation of these molecules within the cell lysosome underlies the pathology of the disease. Each LSD is associated with reduced or ablated expression of a different protein, and exhibits symptoms arising from different organs. Many of these diseases are evident in infancy or childhood, however, some appear later in life. As a group, LSDs occur with incidences of about one in every seven thousand births. While there are no cures for LSDs, intravenous delivery of the deficient enzyme (enzyme replacement therapy, ERT) has been used to ameliorate symptoms in patients with some LSDs. Currently available ERTs do not cross the BBB, therefore, neurological symptoms, such as cognitive decline and behavioral changes, have not been addressed by ERT.