Longitudinal Studies of Brain Structure and Function in MPS Disorders

Oakland Children's Hospital, Oakland, California, United States University of Minnesota, Minneapolis, Minnesota, United States New York University, New York, New York, United States Hospital for Sick Children, Toronto, Ontario, Canada

Neurobehavioral function and quality of life are compromised in many patients with mucopolysaccharidosis (MPS) disorders. The long-term goals of this research are to: 1) more accurately inform patients/parents regarding potential neurobehavioral outcomes; 2) develop sensitive measures of disease progression and central nervous system (CNS) treatment outcome; and 3) help clinical researchers develop direct treatments for specific brain structures/functions. The investigators hypothesize that specific and localized neuroimaging and neuropsychological findings and their relationship will be distinct for each MPS disorder. It is further hypothesized that without treatment, functions will decline and structure will change over time in a predictable fashion, and will be related to locus of abnormality and stage of disease.