



A Natural History Study of the Gangliosidoses

Status: Recruiting

Eligibility Criteria

Age: Not specified

This study is NOT accepting healthy

Healthy Volunteers: volunteers

Inclusion Criteria:

- documented gangliosidosis disease
- *able to complete neuropsychological and neurobehavioral assessments Late-onset gangliosidosis subjects must be able to tolerate MRI of the head

Exclusion Criteria:

- none

Conditions & Interventions

Conditions:

Rare Diseases

Keywords:

GM1 Gangliosidosis, GM2 Gangliosidosis, Sandhoff Disease, Tay-Sachs Disease

More Information

Description: This study's primary aims are to define and characterize disease progression for the infantile and juvenile forms of the gangliosidoses, and the late-onset forms of gangliosidosis, including their heterogeneity; and to observe treatment outcomes for any treatments tried. The secondary aims of this study are to understand the neurological involvement in late-onset gangliosidosis; and to collect data on disease progression that can be used for creation of an objective disease stage and severity index.

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IRB

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