

Synergistic Enteral Regimen for Treatment of the Gangliosidoses (SYNER-G)

Status: Recruiting

Eligibility Criteria

Sex: All

Age: up to 204 Months old

This study is NOT accepting healthy volunteers

Inclusion Criteria:

1. Subjects must have a documented infantile or juvenile gangliosidosis disease. 2. Age: 17 years or less at time of enrollment 3. Subjects and their caregivers must be willing to work with a ketogenic diet team for management of the subject's ketogenic diet.

Exclusion Criteria:

1. A desire to not participate 2. Patients who are older than 17 years will not be enrolled in this study. 3. Children with severe renal impairment will not be enrolled in this study. 4. Post-pubertal females who are pregnant, or who are unwilling to use highly-effective methods to prevent pregnancy, will be excluded from this study. 5. Breast-feeding females will be excluded from this study. 6. Subjects who have an allergy to miglustat or any of the components within the drug product will be excluded from this study.

Conditions & Interventions

Interventions:

Drug: miglustat, Other: Ketogenic Diet

Conditions:

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

Keywords:

infantile Tay-Sachs disease, juvenile Tay-Sachs disease, infantile GM1 gangliosidosis, juvenile GM1 gangliosidosis, infantile GM2 gangliosidosis, juvenile GM2 gangliosidosis, Sandhoff disease, gangliosidoses, miglustat, ketogenic diet, SYNER-G regimen, Syner-G, Zavesca, Tay-Sachs disease, Tay Sachs disease

More Information

Description: The Syner-G regimen research study seeks to evaluate the use of a combination of a medication called miglustat and a ketogenic diet for treatment of the gangliosidoses to learn if this combination will provide improved clinical outcomes compared to what we currently know about the natural course of the disease.

Contact(s): Jeanine James - utzx0002@umn.edu

Principal Investigator: Jeanine James

Phase: Phase 4

IRB Number: 1311M46101

System ID: NCT02030015

Thank you for choosing StudyFinder. Please visit <http://studyfinder.umn.edu> to find a Study which is right for you and contact sfinder@umn.edu if you have questions or need assistance.