

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

Status: Recruiting

Eligibility Criteria

Sex: Male or Female

Age Group: Up to 18 years old

This study is NOT accepting healthy volunteers

Inclusion Criteria:

- no more than 17 years old - documented infantile or juvenile gangliosidosis disease

Exclusion Criteria:

- severe kidney disease - females who are pregnant or breast feeding - females who are post puberty who are unwilling to use highly effective birth control

Conditions & Interventions

Conditions:

Rare Diseases

Keywords:

GM1 Gangliosidoses, GM2 Gangliosidoses, Sandhoff 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.

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Phase: PHASE4

IRB Number: 1311M46101

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