A Phase 1, Open-label, Ascending Dose Study to Evaluate the Safety, Tolerability, Efficacy, Pharmacokinetics and Pharmacodynamics of Recombinant Human Heparan N-Sulfatase (rhHNS, GC1130A) Via Intracerebroventricular Access Device in Patients with Sanfilippo Syndrome Type A (MPS IIIA).

Status: Recruiting

Eligibility Criteria

Sex: Male or Female
Age Group: Up to 18 years old
This study is NOT accepting healthy volunteers

Inclusion Criteria:

- documented MPS IIIA diagnosis - ≥ 24 months and ≤ 72 months of age

Exclusion Criteria:

- significant non-MPS IIIA related central nervous system impairment - previous complication from intraventricular drug administration - contraindications for MRI scans and for neurosurgery - received treatment with any investigational drug or a device intended as a treatment for MPS IIIA within 30 days - received a hematopoietic stem cell or bone marrow transplant or received gene therapy

Conditions & Interventions

Conditions:
Rare Diseases, Rare Diseases
Keywords:
MPS IIIA, Sanfilippo Syndrome

More Information

Description: The purpose of the study is to see if GC1130A, delivered directly to the central ventricle of the brain is safe and tolerable as a means of treating the neurologic disease in MPS 3A.
Study Contact: Melissa Fetterley - melissa.fetterley@fairview.org
Principal Investigator: Chester Whitley, MD, PhD
Phase: PHASE1
IRB Number: STUDY00022733