An Open-label, Phase 1/2 Study to Evaluate the Safety and Efficacy of Single-dose PR001A in Infants with Type 2 Gaucher Disease

Status: Recruiting

Eligibility Criteria

Age: Up to 18 years old

This study is NOT accepting healthy

Healthy Volunteers: volunteers

Inclusion Criteria:

- 0 to 24 months of age - clinical diagnosis on Gaucher disease, Type 2 (GD2) - Bi-allelic GBA1 mutation - child has a reliable caregiver (i.e., parent/legal guardian) who is willing and able to participate in the study as a source of information on the patient's health status and cognitive and functional abilities

Exclusion Criteria:

- diagnosis of a significant CNS disease other than GD2 - able to walk independently - any other significant medical diagnosis (study staff will review) - significant laboratory test result abnormalities - unable to tolerate diagnostic imaging (MRI, CT scan) or unable to tolerate contrast agent - unable to have sedation or anesthesia

Conditions & Interventions

Interventions:
Genetic: LY3884961, Drug: Methylprednisolone, Drug: Prednisone, Drug: Sirolimus
Conditions:
Rare Diseases
Keywords:
Gaucher disease, Type 2 (GD2)

More Information

Description: This is a study to assess the safety and efficacy of PR001A, an Aden-associated (AAV9) viral vector to treat neuronopathic Gaucher disease type 2 (GD2) in infants. PRA001A will be administered via suboccipital injection to the cisterna magna during a single neurosurgical session. GD2 is a fatal disease of early infancy that does not have any therapeutic options beyond palliative care. This study will enroll infants 0-24 months of age.
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Phase: PHASE1
IRB Number: STUDY00008823
System ID: 28776