A Phase 3 Safety and Efficacy Trial of FLT201 Gene Therapy in Patients with Gaucher Disease Type 1

Status: Recruiting

Eligibility Criteria

Sex: Male or Female
Age Group: 18 years and over
This study is NOT accepting healthy volunteers

Inclusion Criteria:

- 18 years of age or older - diagnosed with Gaucher disease type 1 - currently receiving enzyme replacement therapy (ERT) or substrate reduction therapy (SRT) for at least 2 years - see the ClinicalTrials.gov listing for complete inclusion criteria

Exclusion Criteria:

- diagnosis or suspected diagnosis of Gaucher disease type 2 or type 3 - previous gene therapy, cell therapy, or organ/bone marrow transplant - pregnant or breastfeeding - see the ClinicalTrials.gov listing for complete exclusion criteria

Conditions & Interventions

Interventions:
Genetic: FLT201
Conditions:
Rare Diseases, Blood Disorders, Digestive & Liver Health
Keywords:
Clinics and Surgery Center (CSC), Enzyme replacement therapy, ERT, Gaucher disease, Gene therapy, Rare disease

More Information

Description: The purpose of this study is to evaluate the safety and effectiveness of FLT201, an investigational gene therapy for adults with Gaucher disease type 1. Participants will receive a one-time infusion of FLT201, continue their current treatment for a short time, and complete regular study visits and health assessments over a 5-year follow-up period. Researchers will study the safety of FLT201, how well it works, and how long its effects last.
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