

A Gaucher Disease Gene Therapy Trial With FLT201

Status: RECRUITING

Eligibility Criteria

Sex: ALL

Age: 18 years and over

Healthy Volunteers: This study is NOT accepting healthy volunteers

Key Inclusion Criteria:

- Aged ≥ 18 years at time of screening.
- Clinical diagnosis of Gaucher disease type 1
- Stable hemoglobin concentration at baseline
- Stable platelet count at baseline
- Receiving ERT or SRT without interruption for at least 2 years

Key Exclusion Criteria:

- Diagnosed or suspected Gaucher disease type 2 or type 3
- Positive for AAVS3 neutralizing antibodies.
- Abnormal lab values, conditions or diseases that would make the participant unsuitable for the study
- Positive pregnancy test or lactating
- History of hematopoietic stem cell transplant (HSCT)/bone marrow transplant or any solid organ transplant.
- History of receiving any gene therapy or cell therapy.
- History of total splenectomy. Note: Additional protocol defined Inclusion and Exclusion criteria apply

Conditions & Interventions

Interventions:

GENETIC: FLT201

Conditions:

Gaucher Disease Type 1

More Information

Description:

This study is a Phase 3, non-randomized, multicenter, efficacy and safety study in adult patients with Gaucher disease Type 1, on stable treatment with enzyme replacement therapy (ERT) or substrate reduction therapy (SRT) for at least 2 years. The study aims to confirm the efficacy and safety of FLT201 in this population after discontinuation of ERT/SRT.

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Principal Investigator:

Principal Investigator ID:

Phase: PHASE3

IRB Number:

System ID: NCT07223944

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