

A Phase I/II, Multicenter, Open-Label, Single-Dose, Dose-Ranging Study to Assess the Safety and Tolerability of ST-920, a AAV2/6 Human Alpha Galactosidase A Gene Therapy, in Subjects with Fabry Disease

Status: Recruiting

Eligibility Criteria

Sex: Male or Female

Age Group: 18 years and over

This study is NOT accepting healthy volunteers

Inclusion Criteria:

- at least 18 years of age - diagnosis of Fabry disease - one or more of the following symptoms: i) cornea verticillata, ii) acroparesthesia, iii) anhidrosis, iv) angiokeratoma
- fully vaccinated for COVID- 19 per CDC guidance - additional requirements apply for cardiac and renal groups (study staff will review)

Exclusion Criteria:

- history of liver disease - current or history of use in the last six months of systemic steroids - other significant medical & mental health diagnosis (study staff will review)

Conditions & Interventions

Conditions:

Rare Diseases

Keywords:

Fabry Disease

More Information

Description: The purpose of this study is to test a new drug, called ST-920, to see if it is safe and if it works to treat Fabry disease. ST-920 is a gene therapy treatment, which means that ST-920 replaces the missing or broken gene you have because you have Fabry disease, with a version or copy that works.

Study Contact: Brenda Diethelm-Okita - dieth001@umn.edu

Principal Investigator: Chester Whitley, MD, PhD

Phase: PHASE1

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