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

Age: 18 years and over

This study is NOT accepting healthy

Healthy Volunteers: 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 proposed study uses a recombinant AAV2/6 vector encoding the cDNA for human α-Gal A (ST-920). The α-Gal A produced by this cDNA has an identical amino acid sequence to the native enzyme, and also to Fabrazyme® (agalsidase beta or equivalent), a clinically approved recombinant protein product. The ST-920 construct encodes a liver-specific promoter, the human α-1-antitrypsin (hAAT) promoter and includes liver-specific regulatory elements. In addition, rAAV2/6 exhibits liver tropism thus providing the potential for long-term hepatic production of α-Gal A in Fabry disease subjects. Studies of ST-920 in a Fabry disease mouse model administered rAAV2/6 encoding hGLA cDNA by intravenous (IV) injection show generation of therapeutic circulating levels of α-Gal A. The one-time treatment with ST-920 minimizes the risk of infusion--related reactions. The goal of ST-920 is to provide stable, long-term production of α-Gal A at therapeutic levels in subjects with Fabry disease. The constant production of α-Gal A in humans should, importantly, enable reduction and potentially clearance of Fabry disease substrates Gb3 and lyso-Gb3.
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