



MT2024-38: A Phase 1/2 Open-Label, Single-Ascending-Dose Study of EN-374, a Helper-Dependent Adenoviral-Based Gene Therapy, in Participants with X-Linked Chronic Granulomatous Disease

Status: Recruiting

Eligibility Criteria

Sex: Male

Age Group: Not specified

This study is NOT accepting healthy

volunteers

Inclusion Criteria:

- at least 18 years old for initial phase of the study and then at least 3 months old for later phase - diagnosis of X-CGD - history of at least 1 severe infection requiring medical intervention or chronic inflammatory disorder - does not have a suitable, available and willing human related donor - see link to clinicaltrials.gov for complete Inclusion criteria

Exclusion Criteria:

- active infection - history of human immunodeficiency virus (HIV), hepatitis B, or hepatitis C - had investigational gene therapy - see link to clinicaltrials.gov for complete Exclusion criteria

Conditions & Interventions

Conditions:

Rare Diseases

Keywords:

chronic granulomatous disease, recurrent bacterial or fungal infections, X-CGD

More Information

Description: To evaluate the safety of the EN-374 treatment regimen (HSC mobilization, immune prophylaxis, EN-374 dose and administration, and enrichment of HSCs

with O6BG/TMZ).

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Phase: PHASE1

IRB Number: STUDY00025676

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