

Base Editing for Mutation Repair in Hematopoietic Stem & Progenitor Cells for X-Linked Chronic Granulomatous Disease

NCT06325709

Status	RECRUITING
Phase	Phase 1, Phase 2
Sponsor	National Institute of Allergy and Infectious Diseases (NIAID)
Enrollment	10 participants

Key Eligibility Criteria

Inclusion (12)

- ≥ 18 years of age.
 - Confirmed CYBB c.676 C>T mutation.
 - Male patients.
 - Clinically stable and eligible to undergo apheresis and conditioning chemotherapy.
 - $\geq 5 \times 10^6$ cryopreserved cells/kg body weight available for study product manufacturing.
- ... and 7 more (see full listing online)

Exclusion (23)

- Individuals meeting any of the following criteria will be excluded from study participation:
 - Untreated, acute infection.
 - Elevated anti-gp91 specific autoantibodies $> 2 \times$ ULN
 - Elevated anti-gp91 specific T cells (> 10 fold)
 - Anti-platelet antibody screening with > 1 anti-platelet antibody positive in the presence of an ongoing brain infection; OR > 1 anti-platelet antibody positive and considered unsafe for study participation after consultation with hematology specialist.
- ... and 18 more (see full listing online)

Locations (1 total)

National Institutes of Health Clinical Center, Bethesda, Maryland, United States