Allogeneic Hematopoietic Stem Cell Transplantation for Chronic Granulomatous Disease (CGD) With an Alemtuzumab, Busulfan and TBI-based Conditioning Regimen Combined With Cytokine (IL-6, +/- IFN-gamma) Antagonists

NCT05463133

Status	RECRUITING
Phase	Phase 1, Phase 2
Sponsor	National Institute of Allergy and Infectious Diseases (NIAID)
Enrollment	50 participants

Key Eligibility Criteria

Inclusion (16)

- In order to be eligible to participate in this study, an individual must meet all the following criteria:
- Must have the ability to comprehend and a willingness to sign the informed consent. For pediatric patients, must have a parent/guardian who can sign consent if the donor is a minor; assent will be obtained from minors as appropriate.
- Must have confirmed diagnosis of CGD.
- Must have sufficient complications from underlying disease to warrant undergoing transplantation (either a history of or ongoing inflammation/CGD-related autoimmunity OR a CGD-related infection while on prophylaxis) OR have a Quartile 1 or 2 residual oxidase production level.
- Ages 4 years-65 years.

... and 11 more (see full listing online)

Exclusion (13)

- An individual who meets any of the following criteria will be excluded from participation in this study:
- Ejection fraction of less than 30% by echocardiography.
- Forced expiratory volume (FEV1%) of less than 35% and/or an adjusted diffusing capacity of lung of carbon monoxide (adj DLCO) of less than 30%.
- Transaminases \>5x upper limit of normal based on the individual s clinical situation and at the discretion of the investigator.
- Psychiatric disorder or mental deficiency severe enough as to make compliance with the HSCT treatment unlikely, and/or to make regulatorily and legally effective informed consent impossible.

... and 8 more (see full listing online)

Locations (1 total)

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