

Allogeneic Hematopoietic Stem Cell Transplant for GATA2 Mutations

NCT01861106

Status	RECRUITING
Phase	Phase 2
Sponsor	National Cancer Institute (NCI)
Enrollment	144 participants

Key Eligibility Criteria

Inclusion (14)

- ELIGIBILITY CRITERIA:
 - Patient age of 6-70 years.
 - Mutation in the GATA2 gene, or evidence of loss of expression of one allele of GATA2, by cDNA analysis performed by a CLIA certified laboratory, or the clinical syndrome of MonoMAC.
 - Clinical history of at least one serious or disfiguring infection and/or GATA2 bone marrow immunodeficiency disorder with loss of one or more immune populations in the bone marrow including monocytes, Natural Killer (NK) cells, and B-lymphocytes, with or without additional cytopenias involving the red blood cell, neutrophil, or platelet compartment.
 - Availability of a 10/10 or 9/10 or 8/10 HLA-matched related or unrelated donor, or a haploidentical related donor.
- ... and 9 more (see full listing online)

Exclusion (11)

- Patients who are receiving any other investigational agents with the exception of virus- specific cytotoxic T-cells for the treatment of viral infection/reactivation prior to allo HSCT
 - HIV-positive patients are ineligible because these patients are at increased risk of lethal infections when treated with marrow-suppressive therapy. Appropriate studies will be undertaken in patients receiving combination antiretroviral therapy when indicated.
 - History of allergic reactions attributed to compounds of similar chemical or biological composition to agents (steroids, cyclophosphamide, busulfan) used in the study
 - Chronic active hepatitis B. Patient may be hepatitis B core antibody positive. For patients with a concomitant positive hepatitis B surface antigen, patients will require a hepatology consultation. The risk-benefit profile of transplant and hepatitis B will be discussed with the patient, and eligibility determined by the PI or Lead Associate Investigator.
 - History of psychiatric disorder which may compromise compliance with transplant protocol, or which does not allow for appropriate informed consent.
- ... and 6 more (see full listing online)

Locations (1 total)

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

<https://clinicaltrials.gov/study/NCT01861106>

DISCLAIMER: This document is for informational purposes only and does not constitute medical advice. Always consult your healthcare provider before enrolling in any clinical trial. Information may not be up to date — verify details at ClinicalTrials.gov. Generated by ClinicalTrialsFinder.org.