Allogeneic Hematopoietic Stem Cell Transplant for Patients With Inborn Errors of Immunity

NCT04339777

Status	RECRUITING
Phase	Phase 2
Sponsor	National Cancer Institute (NCI)
Enrollment	66 participants

Key Eligibility Criteria

Inclusion (14)

- Age \>= 4 years and \<=69 yo with Weight \>=12 kilograms
- Mutation in a known monogenic (IEI) gene performed by a CLIA certified laboratory, who have failed standard medical management, or when no standard medical management is available.
- OR
- Patients without a known IEI mutation may be eligible if they have a clinical history that is characteristic of an individual with an immune defect including a history of infections requiring prolonged courses of therapy or evidence of immune dysregulation manifested by autoimmune/autoinflammatory disease, atopy, hemophagocytic lymphohistiocytosis, hypogammaglobulinemia, or impaired response to vaccination. A virally-driven malignancy alone will also constitute basis for inclusion.
- Availability of an 8/8, 7/8, or 6/8 HLA-matched related or unrelated donor (if the mismatch is at DQ this will be considered an 8/8 matched donor), or a haploidentical related donor. Karnofsky or Lansky performance status of \>= 40%

... and 9 more (see full listing online)

Exclusion (7)

- Patients who are receiving any other investigational agents (with the exception of virus-specific therapy e.g. cytotoxic T-cells for the treatment of viral infection/reactivation prior to allo HCT).
- Patients with known brain metastases should be excluded from this clinical trial because of their poor prognosis and because they often develop progressive neurologic dysfunction that would confound the evaluation of neurologic and other adverse events.
- 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 biologic composition to agents (steroids, cyclophosphamide, busulfan, tacrolimus, sirolimus, MMF, G-CSF, alemtuzumab) used in the study
- Active psychiatric disorder which is deemed by the PI to have significant risk of compromising compliance with the transplant protocol or which does not allow for appropriate informed consent

... and 2 more (see full listing online)

Locations (1 total)

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