

TCR Alpha Beta T-cell Depleted Haploidentical HCT in the Treatment of Primary Immunodeficiency and Inherited Metabolic Disorders in Children

NCT04414046

Status	RECRUITING
Phase	Phase 2
Sponsor	Johns Hopkins All Children's Hospital
Enrollment	17 participants

Key Eligibility Criteria

Inclusion (15)

- Patient with any form of primary immune deficiency/dysregulatory disorders characterized by aberrant immune function, abnormal hematopoiesis, systemic or organ specific autoimmunity and/or non-malignant lymphoproliferation. This includes, but not limited to:
 - I. Disorders of phagocytes: Chronic granulomatous disease, Leukocyte adhesion deficiency, defects of IL-10 pathway, MonoMac syndrome
 - II. Defects of cellular and humoral immunity: Severe Combined Immunodeficiency Disorder (infants with classic SCID up to 2 years of age will be excluded due to other open protocol), X-linked hyper-IgM syndrome, DOCK8 deficiency, ZAP70 deficiency, common variable immunodeficiency (CVID), Wiskott-Aldrich syndrome, NEMO deficiency.
 - III. Disorder of immune dysregulation: Immunodysregulation polyendocrinopathy enteropathy X-linked (IPEX) syndrome, CTLA4 deficiency, LRBA deficiency, STAT1 GOF, STAT3 GOF, X-linked lymphoproliferative disease etc.
 - IV. Other PIDs and immune dysregulatory disorders who can be benefitted by HCT as deemed appropriate by the PI and the treating immunologist.
- ... and 10 more (see full listing online)

Exclusion (5)

- Participants who have an HLA-matched sibling who is able and willing to donate bone marrow. Patients with a HLA-matched unrelated donors are not excluded.
- Pregnant or breastfeeding females.
- Patient has HIV or uncontrolled fungal, bacterial or viral infections.
- Patient has received prior solid organ transplant.
- Patient has active GVHD (> grade II) or chronic extensive GVHD due to a previous allograft at the time of inclusion.

Locations (1 total)

Johns Hopkins All Children's Hospital, St. Petersburg, Florida, United States