MT2019-09: A randomized trial of low versus moderate exposure busulfan for infants with severe combined immunodeficiency (SCID) receiving TCR alpha beta +/CD19+ depleted transplantation: A Phase II study by the Primary Immune Deficiency Treatment Consortium (PIDTC) and Pediatric Blood and Marrow Transplant Consortium (PBMTC) PIDTC CSIDE Protocol

Status: Recruiting

Eligibility Criteria

Sex: Male or Female
Age Group: Up to 18 years old
This study is NOT accepting healthy volunteers

Inclusion Criteria:

- 0 to 2 years old - infants with SCID, either typical or leaky or Omenn syndrome - see link to clinicaltrials.gov for complete inclusion and exclusion criteria

Exclusion Criteria:

- any serious life-threatening or opportunistic infection at time of enrollment - HIV or HTLV I/II infection

Conditions & Interventions

Conditions:
Cancer, Cancer
Keywords:
SCID, Severe Combined Immunodeficiency

More Information

Description: We want to study if lower doses of a chemotherapy drug called busulfan will help babies with SCID achieve good immunity with less short and long-term risks of complications after transplantation. This trial identifies babies with types of immune deficiencies that are most likely to succeed with this approach and offers them transplant early in life before they get severe infections or later if their infections are under control. It includes only patients receiving unrelated or mismatched related donor transplants.
Study Contact: Andrea Middendorf - midde019@umn.edu
Principal Investigator: Christen Ebens
Phase: PHASE2
IRB Number: SITE00000541