

MT2020-06: A PHASE 1/2 STUDY TO EVALUATE THE SAFETY, TOLERABILITY, AND EFFICACY OF JSP191 FOR HEMATOPOIETIC CELL TRANSPLANTATION CONDITIONING TO ACHIEVE ENGRAFTMENT AND IMMUNE RECONSTITUTION IN SUBJECTS WITH SCID

Status: Recruiting

Eligibility Criteria

Sex: All

Age: 3 Months and over

This study is NOT accepting healthy volunteers

Key

Inclusion Criteria:

All patient groups must have: 1. Typical SCID as defined by Primary Immune Deficiency Treatment Consortia including but not limited to the following subtypes: 1. T-, B+, NK-: IL-2R β deficient, JAK3-deficient 2. T-, B-, NK+: RAG1/2 deficient, Artemis-deficient 3. T-, B+, NK+: IL7R α deficient, CD3 subunit deficient, CD45 deficient OR Variant SCID with absent or low T cell function, Omenn syndrome, Leaky SCID, Reticular dysgenesis, Adenosine deaminase deficiency, and Purine nucleoside phosphorylase deficiency may be included after consultation with the medical monitor. 2. Patients with human leukocyte antigen (HLA) matched related or unrelated donors 3. Adequate end organ function as defined in study protocol

Exclusion Criteria:

1. Patients with any acute or uncontrolled infections 2. Patients receiving any other investigational agents, or concurrent biological, chemotherapy, or radiation therapy 3. Patients with active malignancies 4. Active GVHD within 6 months prior to enrollment, or on immunosuppressive therapy for GVHD

Conditions & Interventions

Interventions:

Biological: Humanized anti-CD117 Monoclonal Antibody (JSP191)

Conditions:

SCID

Keywords:

Immunodeficiency, Pediatric, SCID, Bone Marrow Transplantation, GVHD, Stem Cells, Chimerism, Transplant, BMT

More Information

Description: Phase 1: To evaluate the safety and tolerability of JSP191 and to determine Phase 2 doses of JSP191 as a conditioning agent prior to allogeneic hematopoietic cell transplantation (HCT) in two populations of subjects with severe combined immunodeficiency (SCID): \bullet SCID subjects with history of prior allogeneic HCT but with poor graft function \bullet SCID subjects who are HCT-naïve Phase 2: \bullet To evaluate the efficacy of JSP191 conditioning to enable engraftment of allogeneic CD34+ hematopoietic cells, as determined by CD15+ donor myeloid chimerism \bullet To evaluate the efficacy of JSP191 conditioning to enable immune reconstitution determined by the production of naïve T cells

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Phase: Phase 1/Phase 2

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