Clinical Trial of Upfront Haploidentical or Unrelated Donor BMT to Restore Normal Hematopoiesis in Aplastic Anemia

NCT06517641

Status	RECRUITING
Phase	Phase 2
Sponsor	Medical College of Wisconsin
Enrollment	60 participants

Key Eligibility Criteria

Inclusion (15)

- Age 3 years to 75 years
- Confirmed diagnosis of acquired SAA defined as:
- a. Bone marrow cellularity \< 25% or variable marrow cellularity but with \< 30% residual hematopoietic cells deemed HYPOcellular for age AND b. Two (2) out of 3 of the following (in peripheral blood). i. Neutrophils \< 0.5 x109/L ii. Platelets \< 20 x109/L iii. Reticulocyte count \< 20 x109/L (\< 60 x 109/L using an automated analysis)
- No suitable fully matched related donor as per Investigator's discretion (6/6 match for HLA A and B at intermediate or high-resolution and DRB1 at high-resolution using deoxyribonucleic acid \[DNA\]-based typing) available.
- Available donor as defined in the protocol.

... and 10 more (see full listing online)

Exclusion (16)

- Inherited bone marrow failure syndromes such as Fanconi anemia and short telomere syndromes must be ruled out according to center standards. It is recommended that functional testing for Fanconi Anemia (di-epoxybutane \[DEB\] chromosomal breakage analysis) and telomere length assessment be performed. If available, genetic panels for inherited bone marrow failure syndromes can be considered as an alternative to functional testing.
- Clonal cytogenetic abnormalities consistent with pre-MDS or MDS on marrow examination (e.g., monosomy 7 and other MDS-defining changes per recent pathology guidelines).
- Formal diagnosis of MDS by World Health Organization (WHO) 2022 or International Consensus Classification (ICC).
- Recipient positive for HLA antibodies against a mismatched HLA in the selected donor determined by the presence of donor specific HLA antibodies (DSA) to any mismatched HLA allele/antigen at any of the following loci (HLA-A, -B, -C, -DRB1, DRB3, DRB4, DRB5, -DQA1, -DQB1, -DPA1, -DPB1) with median fluorescence intensity (MFI) \>3000 by microarray-based single antigen bead testing. In patients receiving red blood cell or platelet transfusions, DSA evaluation must be performed or repeated post-transfusion and immediately prior to initiation of recipient preparative regimen to ensure there is confirmation of no DSA to the selected donor when conditioning starts.
- Prior desensitization attempt for HLA antibodies to chosen donor. Any intervention with the sole intent to reduce the level of HLA DSA, (e.g., plasmapheresis, intravenous immunoglobulin \[IVIG\], MMF, etc.) would constitute a desensitization attempt.

... and 11 more (see full listing online)

Locations (25 total)

University of Alabama at Birmingham, Birmingham, Alabama, United States
City of Hope, Duarte, California, United States
University of California, Los Angeles, Los Angeles, California, United States
... and 22 more locations