Unrelated Umbilical Cord Blood Transplantation for Severe Aplastic Anemia and Hypo-plastic MDS Using CordIn(TM), Umbilical Cord Blood-Derived Ex Vivo Expanded Stem and Progenitor Cells to Expedite Engraftment and Improve Transplant Outcome

NCT03173937

Status	RECRUITING
Phase	Phase 1, Phase 2
Sponsor	National Heart, Lung, and Blood Institute (NHLBI)
Enrollment	37 participants

Key Eligibility Criteria

Inclusion (15)

• Diagnosed with severe aplastic anemia with bone marrow cellularity \<30% (excluding lymphocytes) associated with RBC or platelet transfusion dependence and/or neutropenia (absolute neutrophil count \<=1000 cells/ uL or for patients receiving granulocyte transfusions, absolute neutrophil count \<=1000 cells/ uL before beginning granulocyte transfusions).

• OR

• History of severe aplastic anemia transformed to MDS that meet the following criteria: a) International Prognostic Scoring System (IPSS) risk category of INT-1 or greater, b) \< 5% myeloblasts and \< 30% of cellularity in the bone marrow on screening morphologic analysis.

• Intolerance of or failure to respond to immunosuppressive therapy. This also includes patients who have failed immunosuppressive therapy with ATG and cyclosporine or therapy with cyclosporine combined with eltrombopag in those who are intolerant of or do not have access to treatment with ATG.

• Identification of either a) at least one alternative donor (i.e. HLA- haploidentical related donor (i.e. \>=5/10 HLA match: HLA-A, B, C, DR, and DQ loci) or \>=9/10 HLA matched unrelated donor) who is available to serve as a stem cell donor for a salvage allogeneic transplant in the event that the CordIn(TM) unit has been rejected or b) umbilical cord blood unit/s that can be used for a salvage cord blood transplant in the event that the CordIn(TM) unit has been rejected.

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

Exclusion (16)

• Availability of an HLA identical (12/12) matched related or unrelated donor who is available within optimal timeline and suitable considering graft source and established donor selection factors (e.g. age, sex, viral exposure, ABO compatibility, pregnancy status, etc) per PI discretion.

• ECOG performance status of 2 or more.

• Major anticipated illness or organ failure incompatible with survival from transplant.

• Current pregnancy, or unwillingness to take oral contraceptives or use a barrier method of birth control or practice abstinence to refrain from pregnancy, if of childbearing potential for one year.

• HIV positive.

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

Locations (1 total)

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