

MT2013-34C: Hematopoietic Stem Cell Transplant for Dyskeratosis Congenita or Severe Aplastic Anemia: Standard of Care Considerations

Status: Recruiting

Eligibility Criteria

Sex: All

Age: up to 70 Years old

This study is NOT accepting healthy volunteers

Inclusion Criteria:

- Aged 0
- 70 years
- Acceptable hematopoietic stem cell donor
- Dyskeratosis Congenita (DC) with evidence of BM failure defined as:
 - requirement for red blood cell and/or platelet transfusions or
 - requirement for G-CSF or GM-CSF or erythropoietin or
 - refractory cytopenias having one of the following three
 - platelets <50,000/uL or transfusion dependent
 - absolute neutrophil count <500/uL without hematopoietic growth factor support
 - hemoglobin <9g/uL or transfusion dependent
- Diagnosis of DC with a triad of mucocutaneous features:
 - oral leukoplakia
 - nail dystrophy
 - abnormal reticular skin hyperpigmentation, or
- Diagnosis of DC with one of the following:
 - short telomeres (under a research study)
 - mutation in telomerase holoenzyme (DKC1, TERT, TERC, NOP10, NHP2, TCAB1)
 - mutation in shelterin complex (TINF2)
 - mutation in telomere-capping complex (CTC1)
- Severe Aplastic Anemia (SAA) primary transplant with evidence of BM failure:
 - Refractory cytopenia defined by bone marrow cellularity <50% (with < 30% residual hematopoietic cells)
 - Diagnosis of SAA with refractory cytopenias having one of the following three:
 - platelets <20,000/uL or transfusion dependent
 - absolute neutrophil count <500/uL without hematopoietic growth factor support
 - absolute reticulocyte count <20,000/uL
 - Severe Aplastic Anemia (SAA) requiring a 2nd transplant
 - Graft failure as defined by blood/marrow chimerism of < 5%
 - Early myelodysplastic features
 - With or without clonal cytogenetic abnormalities
 - Adequate organ function defined as:
 - cardiac: left ventricular ejection fraction \geq 35% with no evidence of decompensated heart failure
 - pulmonary: DLCO \geq 30% predicted, no supplemental oxygen requirement
 - renal: Glomerular filtration rate (GFR) \geq 30% predicted
- Voluntary written consent

Exclusion Criteria:

- Acute hepatitis or evidence of moderate or severe portal fibrosis or cirrhosis on biopsy
- Pregnant or lactating
- Uncontrolled infection
- Prior radiation therapy (applies to SAA patients only)
- Diagnosis of Fanconi anemia based on DEB
- Diagnosis of dyskeratosis congenita with advanced MDS or acute myeloid leukemia with >30% blasts

Conditions & Interventions

Interventions:

Drug: Alemtuzumab, Drug: Fludarabine, Drug: Cyclophosphamide, Radiation: Total Body Irradiation, Biological: Stem Cell Transplant, Drug: Anti-thymocyte globulin

Conditions:

Dyskeratosis Congenita, Aplastic Anemia

Keywords:

Clinics and Surgery Center (CSC), severe aplastic anemia, Hematopoietic Stem Cell Transplant

More Information

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