HSCT for Patients With Fanconi Anemia Using Risk-Adjusted Chemotherapy

Status: RECRUITING

Eligibility Criteria

Sex: ALL
Age: 3 month(s) and over
Healthy Volunteers: This study is NOT accepting healthy volunteers
Inclusion Criteria:

- Patients must have a diagnosis of Fanconi anemia
- Patients must have one of the following hematologic diagnoses:

1. Severe Aplastic Anemia (SAA), with bone marrow cellularity of <25% OR Severe Isolated Single Lineage Cytopenia and at least one of the following features:
    1. Platelet count <20 x 109/L or platelet transfusion dependence*
    2. ANC <1000 x 109/L
    3. Hgb <8 gm/dl or red cell transfusion dependence*
2. Myelodysplastic Syndrome (MDS) (based on WHO or IPSS Classification
3. Acute Myelogenous Leukemia (untreated, in remission or with refractory or relapsed disease)
    - Donors will be either human leukocyte antigen (HLA) compatible unrelated or HLA-genotypically matched related donors (no fully matched sibling donor).
    - Patients and donors may be of either gender or any ethnic background.
    - Patients must have a Karnofsky adult, or Lansky pediatric performance scale status > 70%.
    - Patients must have adequate physical function measured by:
4. Cardiac: asymptomatic or if symptomatic then 1) left ventricular ejection fraction (LVEF) at rest must be > 50% and must improve with exercise or 2) Shortening Fraction > 29%
5. Hepatic: < 5 x upper limit of normal (ULN) alanine transaminase (ALT) and < 2.0 mg/dl total serum bilirubin.
6. Renal: serum creatinine <1.5 mg/dl or if serum creatinine is outside the normal range, then CrCl > 50 ml/min/1.73 m2
7. Pulmonary: asymptomatic or if symptomatic, DLCO > 50% of predicted
    - Each patient must be willing to participate as a research subject and must sign an informed consent form.
    - Female patients and donors must not be pregnant or breastfeeding at the time of signing consent. Women must be willing to undergo a pregnancy test prior to transplant and avoid becoming pregnant while on study.

Exclusion Criteria:

- Active CNS leukemia
- Female patients who are pregnant (positive serum or urine HCG) or breast-feeding.
- Active uncontrolled viral, bacterial or fungal infection
- Patient seropositive for HIV-I/II; HTLV -I/II

Conditions & Interventions

Interventions:
DRUG: Busulfan, DRUG: Cyclophosphamide, DRUG: Fludarabine, DRUG: rabbit ATG, DRUG: G-CSF, BIOLOGICAL: Peripheral blood stem cell
Conditions:
Fanconi Anemia, Severe Marrow Failure, Myelodysplastic Syndrome (MDS), Acute Myelogenous Leukemia (AML)
Keywords:
marrow aplasia, cytopenia, myelodysplasia, AML, bone marrow transplant, cytoreductive regimen, T-cell reduction

More Information

Description:
The purpose of this study is to determine whether the use of lower doses of busulfan and the elimination of cyclosporine will further reduce transplant-related side effects for patients with Fanconi Anemia (FA). Patients will undergo a transplant utilizing mis-matched related or matched unrelated donors following a preparative regimen of busulfan, fludarabine, anti-thymocyte globulin and cyclophosphamide.

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