STRIDE Biorepository

Status: Recruiting

Eligibility Criteria

Age: 15 years to 40 years old

This study is NOT accepting healthy

Healthy Volunteers: volunteers

Inclusion Criteria:

• Age at least 15 years old to less than 41 years old

• Severe sickle cell disease [any clinically significant sickle genotype, for example, Hemoglobin SS (Hb SS), Hemoglobin SC (Hb SC) or Hemoglobin SBeta thalassemia (Hb S?), or Hemoglobin S-OArab genotype] with at least 1 of the following manifestations: 1. Clinically significant neurologic event (stroke) or any neurological deficit lasting > 24 hours; 2. History of two or more episodes of acute chest syndrome (ACS) in the 2-year period preceding enrollment despite the institution of supportive care measures (i.e. asthma therapy); 3. An average of three or more pain crises per year in the 2-year period preceding enrollment or referral (required intravenous pain management in the outpatient or inpatient hospital setting); 4. Administration of regular red blood cell (RBC) transfusion therapy, defined as receiving 8 or more transfusions per year(in the 12 months before enrollment to prevent vaso-occlusive clinical complications (i.e. pain, stroke, and acute chest syndrome); 5. An echocardiographic finding of tricuspid valve regurgitant jet (TRJ) velocity ? 2.7 m/sec; 6. Ongoing high impact chronic pain on a majority of days per month for at least 6 months.

• Adequate physical function as measured by all of the following: 1. Karnofsky/Lansky performance score > or equal to 60 2. Cardiac function: Left ventricular ejection fraction (LVEF) > 40%; or LV shortening fraction > 26% by cardiac echocardiogram or by Multi Gated Acquisition (MUGA) Scan 3. Pulmonary function: Pulse oximetry with a baseline O2 saturation of ? 85% and diffusing capacity of the lung for carbon monoxide (DLCO) > 40% (corrected for hemoglobin) 4. Renal function: Serum creatinine ? 1.5 x the upper limit of normal for age as per local laboratory and creatinine clearance >70 mL/min; or GFR > 70 mL/min/1.73 m2 by radionuclide Glomerular Filtration Rate (GFR) 5. Hepatic function: Serum conjugated (direct) bilirubin < 2x upper limit of normal for age as per local laboratory; alanine aminotransferase (ALT) and aspartate aminotransferase (AST) < 5 times upper limit of normal as per local laboratory.

Exclusion Criteria:

• Human Leukocyte Antigen (HLA) typing prior to referral (consultation with hematopoietic cell transplantation (HCT) physician). However, if a subject has had HLA typing with accompanying documentation that relatives were not HLA typed and that a search of the unrelated donor registry was not performed the subject will be considered eligible. Documentation will be reviewed and adjudicated by the Protocol Officer or his/her designee.

• Uncontrolled bacterial, viral or fungal infection in the 6 weeks before enrollment.

• Seropositivity for HIV

• Previous HCT or solid organ transplant

• Participation in a clinical trial in which the patient received an investigational drug or device must be discontinued at enrollment.

• A history of substance abuse as defined by version IV of the Diagnostic & Statistical Manual of Mental Disorders (DSM IV).

• Demonstrated lack of compliance with prior medical care (determined by referring physician).

• Pregnant or breast feeding females.

• Inability to receive HCT due to alloimmunization, defined as the inability to receive packed red blood cell (pRBC) transfusion therapy. Additional Eligibility Criteria for Transplant after Biologic Assignment to the Donor Arm: Participants assigned to the Donor Arm at the time of biologic assignment are subject to additional transplant eligibility criteria as specified below. Additional, repeat clinical assessments prior to transplant should be obtained in accordance with institutional policies and standards of care in the interest of good clinical practice.

• Participants must have liver magnetic resonance imaging (MRI) (at least 90 days prior to initiation of transplant conditioning) to document hepatic iron content is required for participants who are currently receiving ?8 packed red blood cell transfusions for ?1 year or have received ?20 packed red blood cell transfusions (cumulative). Participants who have hepatic iron content ?7 mg Fe/g liver dry weight by liver MRI must have a liver biopsy and histological examination/documentation of the absence of cirrhosis, bridging fibrosis, and active hepatitis (at least 90 days prior to initiation of transplant conditioning).

• Cerebral MRI/magnetic resonance angiogram (MRA) within 30 days prior to initiation of transplant conditioning. If there is clinical or radiologic evidence of a recent neurologic event (such as stroke or transient ischemic attack) subjects will be deferred for at least 6 months with repeat cerebral MRI/MRA to ensure stabilization of the neurologic event prior to proceeding to transplantation.

• Documentation of participant's willingness to use approved contraception method until discontinuation of all immunosuppressive medications. This is to be documented in the medical record corresponding with the consent conference.

• Have a suitably matched HLA donor

• Willing and able to donate bone marrow

• Absence of anti-donor HLA antibodies

Conditions & Interventions

Interventions:
Procedure: Blood draw
Conditions:
Anemia, Sickle Cell
Keywords:
Biorepository, Genetics

More Information

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