P3 Study of Umbilical Cord Blood Cells Expanded With MPCs for Transplantation in Patients With Hematologic Malignancies

Purpose

The study investigates the time to engraftment of a mesenchymal expanded cord blood unit in patients with hematologic malignancies undergoing transplantation with myeloablative conditioning.

<table>
<thead>
<tr>
<th>Condition</th>
<th>Intervention</th>
<th>Phase</th>
</tr>
</thead>
<tbody>
<tr>
<td>Acute Myelogenous Leukemia</td>
<td>Biological: Infusion of one MPC expanded cord unit and one unexpanded cord unit</td>
<td>Phase 3</td>
</tr>
<tr>
<td>Acute Lymphoblastic Leukemia</td>
<td></td>
<td></td>
</tr>
<tr>
<td>Non-Hodgkin's Lymphoma</td>
<td></td>
<td></td>
</tr>
<tr>
<td>Hodgkin's Disease</td>
<td>Biological: Infusion of two unexpanded cord blood units.</td>
<td></td>
</tr>
</tbody>
</table>

Study Type: Interventional

Study Design:
- Allocation: Randomized
- Endpoint Classification: Safety/Efficacy Study
- Intervention Model: Parallel Assignment
- Masking: Open Label
- Primary Purpose: Treatment

Official Title: A 1-Year, Multicenter, Randomized, Open-Label Controlled Study to Evaluate the Efficacy and Safety of Cord Blood Cells Expanded With MPCs for Hematopoietic Recovery in Patients With Hematologic Malignancies After Myeloablative Treatment

Resource links provided by NLM:
- Genetics Home Reference related topics: familial acute myeloid leukemia with mutated CEBPA
- MedlinePlus related topics: Acute Myeloid Leukemia, Cancer, Chronic Lymphocytic Leukemia, Hodgkin Disease, Leukemia, Lymphoma
- U.S. FDA Resources

Further study details as provided by Mesoblast, Ltd.:

Primary Outcome Measures:
- Time to Neutrophil and Platelet Engraftment [ Time Frame: 100 days ] [ Designated as safety issue: No ]

Secondary Outcome Measures:
- Proportion of subjects with neutrophil recovery at day 26, platelet recovery at day 60 and subjects alive at day 100 [ Time Frame: 100 days ] [ Designated as safety issue: No ]
Percentage of patients with primary graft failure [Time Frame: 100 days] [Designated as safety issue: Yes]

Other Outcome Measures:
- Incidence and severity of acute Graft Versus Host Disease [Time Frame: 100 days] [Designated as safety issue: Yes]

Estimated Enrollment: 240
Study Start Date: February 2013
Estimated Study Completion Date: July 2018
Estimated Primary Completion Date: February 2018 (Final data collection date for primary outcome measure)

<table>
<thead>
<tr>
<th>Arms</th>
<th>Assigned Interventions</th>
</tr>
</thead>
<tbody>
<tr>
<td>Experimental: Active</td>
<td>Biological: Infusion of one MPC expanded cord unit and one unexpanded cord unit.</td>
</tr>
<tr>
<td>Infusion of one MPC expanded cord unit and one unexpanded cord unit.</td>
<td>Infusion of one MPC expanded cord unit and one unexpanded cord unit.</td>
</tr>
<tr>
<td>Active Comparator: Control</td>
<td>Biological: Infusion of two unexpanded cord blood units.</td>
</tr>
<tr>
<td>Infusion of two unexpanded cord blood units.</td>
<td>Umbilical Cord Blood.</td>
</tr>
</tbody>
</table>

**Eligibility**

Ages Eligible for Study: up to 65 Years
Genders Eligible for Study: Both
Accepts Healthy Volunteers: No

Criteria
Inclusion Criteria:
- Patient must have one of the following:
  - Acute myelogenous leukemia (AML) in complete morphological remission at study screening (Complete Remission with Incomplete Platelet Recovery (CRp) acceptable).
  - Acute lymphoblastic leukemia (ALL) in complete morphological remission at study screening (Complete Remission with Incomplete Platelet Recovery (CRp) acceptable).
  - Non-Hodgkin's lymphoma (NHL): High risk subjects with responsive disease after first relapse. High risk includes those with Burkitt's Lymphoma and those with extensive marrow involvement at diagnosis-precluding autologous transplant.
  - Hodgkin's disease: High risk subjects with responsive disease after first relapse.
- Minimum Karnofsky Scale
- Subject must weigh at least 20 kg
- Up to 65 years of age
- Adequate major organ system function

Exclusion Criteria:
- Pregnancy and/or lactating
- Suitable, 6/6 HLA matched related sibling donor available
- Previous participation in a stem cell study within last 30 days

**Contacts and Locations**

Please refer to this study by its ClinicalTrials.gov identifier: NCT01854567

Contacts
Contact: Rebecca Cohen rebecca.cohen@mesoblast.com

Locations
United States, Florida
Moffitt Cancer Center Tampa, Florida, United States, 33612

Recruiting
Principal Investigator: Marcie Tomblyn, MD

**United States, New York**

Well Cornell-New York Presbyterian Hospital
New York, New York, United States, 10065
Principal Investigator: Tsiporah B Shore, MD

Westchester Medical Center
Valhalla, New York, United States, 10595
Principal Investigator: Mitchell Cairo, MD

**United States, Ohio**

Case Western
Cleveland, Ohio, United States, 44106
Principal Investigator: Hiliard Lazarus, MD
Sub-Investigator: Marcos deLima, MD

**United States, Texas**

MD Anderson Cancer Center
Houston, Texas, United States, 77030
Principal Investigator: Elizabeth J Shpall, MD

Texas Transplant Center at Methodist Healthcare System
San Antonio, Texas, United States, 78229
Principal Investigator: Paul Shaughnessy, MD

**Sponsors and Collaborators**

Mesoblast, Ltd.

**Investigators**

Study Director: Donna Skerrett, MD, MS Mesoblast, Ltd.
Principal Investigator: Elizabeth J. Shpall, MD M.D. Anderson Cancer Center

**More Information**

No publications provided

Responsible Party: Mesoblast, Ltd.
ClinicalTrials.gov identifier: NCT01854567 History of Changes
Other Study ID Numbers: CB-AB006, 2012-0166
Study First Received: May 13, 2013
Last Updated: August 22, 2013
Health Authority: United States: Food and Drug Administration

Keywords provided by Mesoblast, Ltd.:

**Cord Blood**
AML
ALL
MPC
NHL
Mesoblast
Leukemia
Expanded
Lymphoma

Additional relevant MeSH terms:

**Hodgkin Disease**
Leukemia, Myeloid, Acute
Leukemia, Myeloid
Lymphoma

**Lymphoproliferative Disorders**
Leukemia, Non-Hodgkin
Hematologic Neoplasms

**Lymphatic Diseases**
Neoplasms by Histologic Type
Neoplasms

**Immunoproliferative Disorders**
Neoplasms by Site

**Immune System Diseases**

**Hematologic Diseases**

**Leukemia**

**Leukemia, Lymphoid**

**Precursor Cell Lymphoblastic Leukemia-Lymphoma**

ClinicalTrials.gov processed this record on September 22, 2013