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Trial record **7 of 9** for: Hurler's Syndrome and cord blood
Previous Study | Return to List | Next Study

Stem Cell Transplantation for Hurler

This study has been completed.

Sponsor:
Masonic Cancer Center, University of Minnesota

Information provided by (Responsible Party):
Masonic Cancer Center, University of Minnesota

ClinicalTrials.gov Identifier:
NCT00176917

First received: September 12, 2005
Last updated: November 6, 2012
Last verified: November 2012
History of Changes

Full Text View | Tabular View | Study Results | Disclaimer | How to Read a Study Record

Purpose

The purpose of this study is to determine the safety and engraftment of donor hematopoietic cells using this conditioning regimen in patients undergoing a hematopoietic (**blood** forming) cell transplant for **Hurler syndrome**, Maroteaux Lamy **syndrome**, Mannosidosis, or I-cell disease.

Condition	Intervention	Phase
Mucopolysaccharidosis I Mucopolysaccharidosis VI Mannosidosis Mucolipidosis Type II (I-cell Disease)	Procedure: Stem Cell Transplant Drug: Busulfan, Cyclophosphamide, ATG	Phase 2

Study Type: Interventional
Study Design: Endpoint Classification: Efficacy Study
Intervention Model: Single Group Assignment
Masking: Open Label
Primary Purpose: Treatment

Official Title: Hematopoietic Stem Cell Transplantation for **Hurler Syndrome**, Maroteaux Lamy **Syndrome** (MPS VI), and Alpha Mannosidase Deficiency (Mannosidosis)

Resource links provided by NLM:

Genetics Home Reference related topics: alpha-mannosidosis mucolipidosis II alpha/beta mucopolysaccharidosis type I mucopolysaccharidosis type VI Schindler disease succinic semialdehyde dehydrogenase deficiency

Drug Information available for: Cyclophosphamide Busulfan

U.S. FDA Resources

Further study details as provided by Masonic Cancer Center, University of Minnesota:

Primary Outcome Measures:

- Mean Percentage of Donor Cells in Study Population (Chimerism). [Time Frame: at 21 days, 42 days, 60 days, 100 days, 6 months, and 1 year] [Designated as safety issue: No]

  Donor-derived engraftment determined by restriction fragment length polymorphism (RFLP).

Secondary Outcome Measures:

- Number of Patients Surviving on Study [Time Frame: at 100 days, 1 year, and 3 years post transplant] [Designated as safety issue: Yes]

Number of patients surviving (alive) at specified timepoints.

- Number of Patients Who Failed Engraftment. [Time Frame: Day 42 Post Transplant] [Designated as safety issue: Yes]

  Toxicity (undesireable effect) of hematologic donor cell engraftment is determined by failure to engraft at Day 42.

- Number of Patients With Grade III-IV Acute Graft-versus-host Disease (aGVHD). [Time Frame: Day 100 Post Transplant] [Designated as safety issue: Yes]

  Toxicity (undesireable effect) of this stem cell transplant preparative regimen due to acute graft-versus-host disease.

Enrollment: 41
Study Start Date: May 1999
Study Completion Date: May 2010
Primary Completion Date: May 2008 (Final data collection date for primary outcome measure)

Arms	Assigned Interventions
Experimental: Treatment Arm All patients treated with chemotherapy and transplantation.	Procedure: Stem Cell Transplant The purpose of hematopoietic cell transplantation is to introduce hematopoietic cells from a normal donor that contains the enzyme able to get rid of the substances that have accumulated in the body of patients with storage diseases. Hematopoietic cells can come from bone marrow, peripheral **blood** (i.e., the **blood** circulating in our body's **blood** vessels) or umbilical **cord blood** (i.e. **blood** taken from the umbilical **cord** after a baby is born and umbilical **cord** is cut). Other Name: Bone Marrow Transplant Drug: Busulfan, Cyclophosphamide, ATG Prior to transplantation, subjects will receive BUSULFAN intravenously (IV) via the Hickman line twice daily for 4 days, CYCLOPHOSPHAMIDE intravenously via the Hickman line once a day for 4 days, and ANTI-THYMOCYTE GLOBULIN IV via the Hickman line twice daily for three days before the transplant. These three drugs are being given to help the new marrow "take" and grow. METHYLPREDNISOLONE will be given as a pre-medication for the ATG. Other Name: Busulfex, Cytoxan, Thymoglobulin

Detailed Description:
Prior to transplantation, subjects will receive Busulfan intravenously (IV) via the Hickman line four times daily for 4 days, Cyclophosphamide intravenously via the Hickman line once a day for 4 days, and Anti-Thymocyte Globulin IV via the Hickman line twice daily for three days before the transplant. These three drugs are being given to subjects to help the new marrow "take" and grow.

On the day of transplantation, the donor's hematopoietic cells will be transfused via central venous catheter.

After hematopoietic cell transplant, subjects will then receive two drugs, cyclosporin and either methylprednisolone or Mycophenolate Mofetil (MMF). Cyclosporin and methylprednisolone or MMF are given to help prevent the complication of graft-versus-host disease and to decrease the chance that the new donor cells will be rejected.

Eligibility

Genders Eligible for Study: Both
Accepts Healthy Volunteers: No

Criteria
Inclusion Criteria:

- Patients with Mucopolysaccharidosis, type I (e.g., Hurler syndrome), Maroteaux-Lamy syndrome (MPS VI), Alpha Mannosidosis, or mucolipidosis type II (I-cell disease) who have an HLA-identical or mismatched (at 1 antigen) related marrow, PBSC, or cord blood donor.
- Patients with Mucopolysaccharidosis, type I, Maroteaux-Lamy syndrome (MPS VI), Alpha Mannosidosis, or mucolipidosis type II (I-cell disease) who have an HLA-identical or HLA-1 antigen mismatched unrelated marrow, PBSC, or HLA-0-2 antigen mismatched umbilical cord blood donor.
- Patients with MPS type I, Maroteaux Lamy Syndrome (MPS VI), or mucolipidosis type II (I-cell disease) will have a mental developmental index within two standard deviations of the normal mean, as best as can be determined using Bayley scales of infant development or other standardized testing, recognizing that these may be affected by speech and/or hearing impairment.
- Adequate organ function:
- Cardiac: ejection fraction >40%; no decompensated congestive heart failure or uncontrolled arrhythmia
- Renal: serum creatinine <2.0 mg/dl
- Hepatic: total bilirubin <3x Upper limits of normal transaminases < 5.0 x Upper limits of normal
- Signed consent.

Exclusion Criteria:

- Presence of major organ dysfunction (see above)
- Pregnancy
- Evidence of HIV infection or known HIV positive serology
- Patients or parents are psychologically incapable of undergoing BMT with associated strict isolation or documented history of medical non-compliance
- Patients >50 kg may be at risk for having cell doses below the goal of ≥ 10 x 106 CD 34 cells/kg and therefore will not be eligible to receive unrelated PBSCs.

Contacts and Locations

Please refer to this study by its ClinicalTrials.gov identifier: NCT00176917

Locations

United States, Minnesota

Masonic Cancer Center, University of Minnesota
Minneapolis, Minnesota, United States, 55455

Sponsors and Collaborators

Masonic Cancer Center, University of Minnesota

Investigators

Principal Investigator: Paul Orchard, MD Masonic Cancer Center, University of Minnesota

More Information

No publications provided

Responsible Party:	Masonic Cancer Center, University of Minnesota
ClinicalTrials.gov Identifier:	NCT00176917 History of Changes
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Study First Received:	September 12, 2005
Results First Received:	July 28, 2009
Last Updated:	November 6, 2012
Health Authority:	United States: Institutional Review Board

Keywords provided by Masonic Cancer Center, University of Minnesota:
stem cell transplant
storage disease
errors of metabolism

Additional relevant MeSH terms:

Mucolipidoses	Metabolism, Inborn Errors
Mucopolysaccharidosis I	Genetic Diseases, Inborn
Alpha-Mannosidosis	Carbohydrate Metabolism, Inborn Errors
Mannosidase Deficiency Diseases	Lysosomal Storage Diseases
Mucopolysaccharidoses	Metabolic Diseases
Mucopolysaccharidosis VI	Mucinoses
Bone Diseases, Metabolic	Connective Tissue Diseases
Bone Diseases	Busulfan
Musculoskeletal Diseases	Cyclophosphamide
Lysosomal Storage Diseases, Nervous System	Immunosuppressive Agents
Brain Diseases, Metabolic, Inborn	Immunologic Factors
Brain Diseases, Metabolic	Physiological Effects of Drugs
Brain Diseases	Pharmacologic Actions
Central Nervous System Diseases	Antineoplastic Agents, Alkylating
Nervous System Diseases	Alkylating Agents

ClinicalTrials.gov processed this record on September 22, 2013