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Trial record **3 of 14** for: Sickle Cell Anemia and umbilical cord blood

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Cord Blood Transplantation for Sickle Cell Anemia and Thalassemia

This study has been completed.

Sponsor:
National Heart, Lung, and **Blood** Institute (NHLBI)

Information provided by:
National Heart, Lung, and Blood Institute (NHLBI)

ClinicalTrials.gov Identifier:
NCT00029380

First received: January 10, 2002
Last updated: September 30, 2008
Last verified: September 2008
History of Changes

Full Text View | **Tabular View** | **No Study Results Posted** | Disclaimer | How to Read a Study Record

Purpose

This study will develop a national **cord blood** bank for siblings of patients with hemoglobinopathies and thalassemia.

Condition	Intervention	Phase
Hematologic Diseases **Anemia**, **Sickle Cell** Beta-Thalassemia Hematopoietic Stem **Cell** Transplantation	Drug: Sangstat Drug: Cyclophosphamide Drug: Busulfan Drug: Mycophenolate Mofetil Drug: Cyclosporine Procedure: **Cord Blood** Transplantation	Phase 2

Study Type: Interventional
Study Design: Primary Purpose: Treatment

Official Title: Sibling Donor **Cord Blood** Banking and Transplantation

Resource links provided by NLM:

Genetics Home Reference related topics: beta thalassemia sickle cell disease

MedlinePlus related topics: Anemia Blood Disorders Sickle Cell Anemia Thalassemia

Drug Information available for: Cyclophosphamide Busulfan Mycophenolic acid Mycophenolate sodium Cyclosporine Mycophenolate mofetil hydrochloride Mycophenolate mofetil

U.S. FDA Resources

Further study details as provided by National Heart, Lung, and Blood Institute (NHLBI):

Primary Outcome Measures:

- Hematologic parameters
- GVHD

Estimated Enrollment: 30
Study Start Date: January 1999
Study Completion Date: August 2006
Primary Completion Date: August 2006 (Final data collection date for primary outcome measure)

Detailed Description:
BACKGROUND:

During the past decade, a number of advances have been made in the treatment of patients with sickle cell anemia and thalassemia. Among these advances is allogeneic bone marrow transplantation, which is the only current treatment that offers a potential for cure. In sickle cell anemia, transplantation has been performed in patients who have had advanced organ damage. In thalassemia, transplantation has been performed before having any evidence of iron-related tissue damage. Due to concerns over engraftment and graft versus host disease (GVHD), transplants for patients with hemoglobinopathies have been limited to situations in which a human leukocyte antigen (HLA) compatible donor existed. Unfortunately, an HLA-matched related donor is often not available. Umbilical cord blood (UCB), a recently recognized source of hematopoietic stem cells, has been used to successfully transplant bone marrow to over 500 patients. The potential advantage of cord blood over other donor sources of stem cells is the minimal risk of high-grade GVHD (even without complete HLA compatibility).

DESIGN NARRATIVE:

This study will establish a national sibling donor cord blood (SDCB) program, evaluate its use in a multi-center pilot study of transplantation, and develop a Web-based data management system to support these two projects. A multi-center pilot study was conducted on cord blood transplantation in children with either sickle cell disease or thalassemia. The investigators tested the hypothesis that a novel immunosuppressive conditioning regimen (fludarabine, cyclophosphamide, and busulfan) and post transplant therapy (mycophenolate mofetil and cyclosporine) would improve engraftment rates and prevent disease recurrence. The effect of SDCB transplantation on hematologic parameters and GVHD was monitored. Enrollment in the study was suspended on December 29, 2003. The protocol was revised, replacing the previous conditioning regimen of fludarabine, busulfan, and cyclophosphamide with a more conventional regimen of rabbit anti-thymocyte globulin (Sangstat), busulfan, and cyclophosphamide. The revised protocol is open for enrollment.

Eligibility

Ages Eligible for Study:	3 Years to 14 Years
Genders Eligible for Study:	Both
Accepts Healthy Volunteers:	No

Criteria
Inclusion Criteria:

- Suitable UCB collection from an HLA-identical sibling
- Sickle cell anemia (Hb SS or S beta thalassemia) with significant disease manifestations as defined by at least one of the following criteria:
    a. A history of painful events defined as three or more painful events in the 2 years prior to enrollment. Pain may occur in typical sites associated with vaso-occlusive painful events and cannot be explained by causes other than sickle cell disease. The pain must last at least 4 hours and require treatment with either parenteral narcotics, an equianalgesic dose of oral narcotics (if pain is treated in a local facility where parenteral narcotics are not routinely used to treat painful events), or parenteral nonsteroidal anti-inflammatory drugs. Painful events managed at home will be considered only if there is documentation of the event in a clinical record that may be reviewed by an investigator.
    b. Acute chest syndrome (ACS) with two or more episodes of ACS with the development of a new infiltrate on chest radiograph and/or having a perfusion defect demonstrable on a lung radioisotope scan
    c. Any combination of painful events and episodes of ACS that total three events in the 2 years before transplantation
    d. Any clinically significant neurologic event (stroke or hemorrhage) or any neurologic defect lasting more than 24 hours
    e. Abnormal cerebral MRI and abnormal cerebral MRA
    f. An episode of dactylitis in the first year of life with significant anemia (Hbg less than 7 g/dL), or leukocytosis in the second year of life such that the risk of a severe adverse outcome before 18 years of age exceeds 54% (as defined by the cooperative study of sickle cell disease (CSSCD) infant cohort study)
    g. History of positive trans-cranial Doppler studies (average greater than 200 cm/sec)
- Beta thalassemia major with significant disease manifestations as defined by the following criteria: Beta thalassemia genotype consistent with clinical diagnosis of beta thalassemia major (could include patients with E-beta thalassemia genotype) and requiring eight or more red blood cell (RBC) transfusions a year and iron chelation therapy. Younger patients who are at risk of transfusional iron overload but who have not yet initiated iron chelation therapy will be eligible.
- Adequate physical function as measured by the following criteria:
    a. Cardiac: Asymptomatic or, if symptomatic, then left ventricular ejection fraction at rest must be greater than 40% and must improve with exercise, or shortening fraction greater than 26%
    b. Hepatic: Less than 5 times the clinical baseline of AST and less than 2.5 times the clinical baseline mg/dL of total serum bilirubin (clinical baseline is determined from the mean of the four most recent test results)
    c. Renal: Serum creatinine within normal range for age or if serum creatinine is outside normal range for age then renal function (creatinine clearance or GFR) greater than 50% of the lower limit of normal (LLN) for age
    d. Pulmonary: Asymptomatic, or, if symptomatic, DLCO, FEV1, FEC (diffusion capacity) greater than 45% of predicted (corrected for hemoglobin); if unable to obtain PFT, oxygen saturation greater than 85% on room air

Contacts and Locations

Please refer to this study by its ClinicalTrials.gov identifier: NCT00029380

Locations

United States, California

Children's Hospital, Oakland
Oakland, California, United States, 94609

Children's Hospital Oakland
Oakland, California, United States, 94609

United States, District of Columbia

Children's National Medical Center
Washington, District of Columbia, United States

United States, Florida

Nemours Children's Clinic
Jacksonville, Florida, United States, 32207

University of Miami Batchelor Children's Research Center
Miami, Florida, United States, 33136

United States, Illinois

Children's Memorial Hospital
Chicago, Illinois, United States, 60614

United States, Louisiana

Louisiana State University Children's Medical Center
New Orleans, Louisiana, United States

United States, Michigan

University of Michigan
Ann Arbor, Michigan, United States, 48109

United States, New Jersey

Hackensack University Medical Center
Hackensack, New Jersey, United States, 07601

United States, North Carolina

Duke University Medical Center Children's Hospital
Durham, North Carolina, United States

United States, Pennsylvania

Children's Hospital Philadelphia
Philadelphia, Pennsylvania, United States, 19104

United States, South Carolina

Medical University of South Carolina
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Sponsors and Collaborators

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Investigators

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Study Chair:	Donna Wall	Texas Transplant Institute
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More Information

Publications:

Reed W, Walters M, Lubin BH. Collection of sibling donor cord blood for children with thalassemia. J Pediatr Hematol Oncol. 2000 Nov-Dec;22(6):602-4.

Lubin BH, Eraklis M, Apicelli G. Umbilical cord blood banking. Adv Pediatr. 1999;46:383-408. Review. No abstract available.

Woodard P, Lubin B, Walters CM. New approaches to hematopoietic cell transplantation for hematological diseases in children. Pediatr Clin North Am. 2002 Oct;49(5):989-1007. Review.

Reed W, Smith R, Dekovic F, Lee JY, Saba JD, Trachtenberg E, Epstein J, Haaz S, Walters MC, Lubin BH. Comprehensive banking of sibling donor cord blood for children with malignant and nonmalignant disease. Blood. 2003 Jan 1;101(1):351-7.

Locatelli F, Rocha V, Reed W, Bernaudin F, Ertem M, Grafakos S, Brichard B, Li X, Nagler A, Giorgiani G, Haut PR, Brochstein JA, Nugent DJ, Blatt J, Woodard P, Kurtzberg J, Rubin CM, Miniero R, Lutz P, Raja T, Roberts I, Will AM, Yaniv I, Vermylen C, Tannoia N, Garnier F, Ionescu I, Walters MC, Lubin BH, Gluckman E. Related umbilical cord blood transplant in patients with Thalassemia and Sickle Cell Disease. Blood. 2002 Nov 7 [epub ahead of print]

Reed W, Walters M, Trachtenberg E, Smith R, Lubin BH. Sibling donor cord blood banking for children with sickle cell disease. Pediatr Pathol Mol Med. 2001 Mar-Apr;20(2):167-74.

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Additional relevant MeSH terms:

Anemia
Anemia, **Sickle Cell**
Anemia, Hemolytic, Congenital
Anemia, Hemolytic
Beta-Thalassemia
Hematologic Diseases
Thalassemia
Hemoglobinopathies
Genetic Diseases, Inborn
Busulfan
Cyclophosphamide
Cyclosporins
Cyclosporine
Mycophenolate mofetil
Mycophenolic Acid
Immunosuppressive Agents
Immunologic Factors
Physiological Effects of Drugs
Pharmacologic Actions
Antineoplastic Agents, Alkylating
Alkylating Agents
Molecular Mechanisms of Pharmacological Action
Antineoplastic Agents
Therapeutic Uses
Myeloablative Agonists
Antirheumatic Agents
Enzyme Inhibitors
Antifungal Agents
Anti-Infective Agents
Dermatologic Agents

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