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Trial record **2 of 13** for: thalassemia 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

Tracking Information	
First Received Date ICMJE	January 10, 2002
Last Updated Date	September 30, 2008
Start Date ICMJE	January 1999
Primary Completion Date	August 2006 (final data collection date for primary outcome measure)
Current Primary Outcome Measures ICMJE **(submitted: April 27, 2006)**	• Hematologic parameters • GVHD
Original Primary Outcome Measures ICMJE	*Not Provided*
Change History	Complete list of historical versions of study NCT00029380 on ClinicalTrials.gov Archive Site
Current Secondary Outcome Measures ICMJE	*Not Provided*
Original Secondary Outcome Measures ICMJE	*Not Provided*
Current Other Outcome Measures ICMJE	*Not Provided*
Original Other Outcome Measures ICMJE	*Not Provided*

Descriptive Information	
Brief Title ICMJE	**Cord Blood** Transplantation for Sickle Cell Anemia and **Thalassemia**
Official Title ICMJE	Sibling Donor **Cord Blood** Banking and Transplantation
Brief Summary	This study will develop a national **cord blood** bank for siblings of patients with hemoglobinopathies and **thalassemia**.
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.
Study Type ICMJE	Interventional
Study Phase	Phase 2
Study Design ICMJE	Primary Purpose: Treatment
Condition ICMJE	• Hematologic Diseases • Anemia, Sickle Cell • Beta-**Thalassemia** • Hematopoietic Stem Cell Transplantation
Intervention ICMJE	• Drug: Sangstat • Drug: Cyclophosphamide • Drug: Busulfan • Drug: Mycophenolate Mofetil • Drug: Cyclosporine • Procedure: **Cord Blood** Transplantation
Study Arm (s)	*Not Provided*
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.

*** Includes publications given by the data provider as well as publications identified by ClinicalTrials.gov Identifier (NCT Number) in Medline.**

Recruitment Information

Recruitment Status ICMJE	Completed
Estimated Enrollment ICMJE	30
Completion Date	August 2006
Primary Completion Date	August 2006 (final data collection date for primary outcome measure)
Eligibility Criteria ICMJE	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
Gender	Both
Ages	3 Years to 14 Years

Accepts Healthy Volunteers	No
Contacts ICMJE	*Contact information is only displayed when the study is recruiting subjects*
Location Countries ICMJE	United States, Canada

Administrative Information

NCT Number ICMJE	NCT00029380
Other Study ID Numbers ICMJE	141, U01 HL61877
Has Data Monitoring Committee	*Not Provided*
Responsible Party	Bertram H. Lubin, Children's Hospital, Oakland
Study Sponsor ICMJE	National Heart, Lung, and **Blood** Institute (NHLBI)
Collaborators ICMJE	*Not Provided*
Investigators ICMJE	

Study Chair:	Victor Aquino	University of Texas Southwestern Medical Center - Dallas	
Study Chair:	Nancy Bunin	Children's Hospital Philadelphia	
Study Chair:	Martin Champagne	Hopital Ste-Justine	
Study Chair:	Joel Brochstein	Hackensack University Medical Center	
Study Chair:	Michael Joyce	Nemours Children's Clinic	
Study Chair:	Naynesh Kamani	Children's Research Institute	
Study Chair:	Gary Kleiner	University of Miami Batchelor Children's Research Center	
Study Chair:	Joanne Kurtzberg	Duke University Medical Center Children's Hospital	
Study Chair:	Bertram H. Lubin	Children's Hospital & Research Center Oakland	
Study Chair:	Alexis Thompson	Ann & Robert H Lurie Children's Hospital of Chicago	
Study Chair:	Donna Wall	Texas Transplant Institute	
Study Chair:	Mark Walters	Children's Hospital & Research Center Oakland	
Study Chair:	Lolie Yu	Louisiana State University Children's Medical Center	

Information Provided By	National Heart, Lung, and Blood Institute (NHLBI)
Verification Date	September 2008

ICMJE **Data element required by the International Committee of Medical Journal Editors and the World Health Organization ICTRP**