Collection and Storage of Umbilical Cord Stem Cells for Treatment of Sickle Cell Disease

Purpose

This study will determine the best ways to collect, process and store umbilical cord blood from babies with sickle cell disease, sickle cell trait and unaffected babies. Sickle cell disease is an abnormality of the hemoglobin in red blood cells that causes the cells to change shape and clump together, preventing their normal flow in the bloodstream. This impairs blood flow to various organs, and the resulting oxygen deprivation causes organ damage.

Cord blood is rich in stem cells (cells produced in the bone marrow that mature to different types of blood cells), which may prove useful in new sickle cell therapies. However, cord blood from babies with sickle cell trait, sickle cell disease and normal babies may act differently under laboratory conditions, so it is important to learn how best to work with blood from all three groups of babies for future use in possible treatments.

Pregnant women between 18 and 45 years of age who are at risk of having an infant with sickle cell disease and normal volunteers who are pregnant and not at risk for this disease may be eligible for this study. Potential participants will be counseled about donating her infant’s blood in order to make an informed choice.

All women who participate in the study will provide a medical history and have blood collected from the umbilical cord and placenta (afterbirth) after the baby’s delivery. The blood will be tested for various infectious diseases, processed, frozen and stored for research purposes. In addition, blood from women with babies at risk for sickle cell disease will be tested for the presence of the sickle cell gene, tissue typed, and used for research as follows:

- **Sickle cell disease** - If cord blood tests show the baby has sickle cell disease, the blood will be frozen for an indefinite period of time for possible use in future treatment of the child. This treatment could include stem cell transplantation or gene therapy, treatments are not currently considered routine for sickle cell disease.

- **Sickle cell trait or normal hemoglobin** - If cord blood tests show the baby has sickle cell trait or is unaffected, the blood will be processed and stored for up to 3 years, during which time it may possibly be used to treat a currently living or future sibling with sickle cell disease. After 3 years, the participant may agree to either have the blood discarded, given to research or moved to another facility for continued storage at the participant’s expense. Alternatively, if there is no anticipated future need for the collected blood, or if it does not meet standards needed for future treatment, it will be used in NIH-approved research studies.

Participants and their family doctor or the baby’s pediatrician will be contacted twice a year for information about changes in the baby’s health. Participants may also be asked permission to perform new tests developed by researchers.

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<th>Condition</th>
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<tr>
<td>Healthy</td>
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<td>Sickle Cell Anemia</td>
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Study Type: Observational

Official Title: Collection and Storage of Umbilical Cord Hematopoietic Stem Cells for Sickle Cell Disease Therapy

Resource links provided by NLM:

- Genetics Home Reference related topics: sickle cell disease
- MedlinePlus related topics: Anemia, Sickle Cell Anemia
- U.S. FDA Resources

Further study details as provided by National Institutes of Health Clinical Center (CC):

Estimated Enrollment: 99999999
Study Start Date: March 2001

Detailed Description:
Umbilical cord blood is a source of hematopoietic stem cells (HSCs) for transplantation or gene therapy. Our goal is to procure umbilical cord blood (UCB) from newborns at risk for sickle cell disease, sickle cell trait, and related disorders as well as normal newborns, for our controls, in order to develop methods for processing and cryopreservation of umbilical cord blood HSCs for use in future clinical transplantation or gene therapy. In order to carry out our methods development research umbilical cord blood units will be collected from an indefinite number of subjects until 30 cord blood units from newborns with sickle cell disease have been cryopreserved. These units will be stored for future gene therapy. Maternal subjects will have been identified as being at risk to have an infant with sickle cell disease, will be between the ages of 18 and 45, and will meet specified medical history criteria. The cord blood units will be tested for transfusion transmissible viruses, infectious disease markers, Human Leukocyte Antigen (HLA) typing, Hemoglobin genotyping, and enumeration of progenitor cells. The umbilical cord blood units will be used for the developmental research on processing/cryopreservation methods but, once processed and stored, may also be identified for future clinical use or for basic or translational research by NIH investigators. This study will be a multisite collaboration with Washington metropolitan area hospitals.

**Eligibility**

Ages Eligible for Study: 18 Years to 45 Years  
Genders Eligible for Study: Female  
Accepts Healthy Volunteers: Yes

**Criteria**

- **INCLUSION CRITERIA:**  
  Pregnant women who are at risk of having an infant with sickle cell anemia (HbSS), as well as woman who are not at risk and wish to serve as control subjects, will be identified and referred by their health care providers or will be self-referred.  
  Maternal subjects must be between 18 and 45 years old, may be in their first or subsequent pregnancy, and must be able to provide informed consent.

- **EXCLUSION CRITERIA:**  
  The maternal subject will not be eligible for study if she is known to be positive for one or more of the following diseases transmissible by blood: HIV, hepatitis B, hepatitis C, or HTLV; is unable to give informed consent; or is known to have a fetus with a significant congenital anomaly.  
  Subjects may be excluded at the time of delivery if the attending physician or collection staff, due to unanticipated obstetrical complications, deems cord blood collection inadvisable.

**Contacts**

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Contact: John F Tisdale, M.D.  
(301) 402-6497  johntis@mail.nih.gov

**Locations**

United States, Maryland  
National Institutes of Health Clinical Center, 9000 Rockville Pike  
Bethesda, Maryland, United States, 20892  
Contact: For more information at the NIH Clinical Center contact Patient Recruitment and Public Liaison Office (PRPL)  
800-411-1222 ext TTY8664111010

**Sponsors and Collaborators**

National Heart, Lung, and Blood Institute (NHLBI)

**Investigators**

Principal Investigator: John F Tisdale, M.D.  National Heart, Lung, and Blood Institute (NHLBI)

**More Information**

Additional Information:  
[NIH Clinical Center Detailed Web Page](https://clinicaltrials.gov/ct2/show/NCT00012545?term=Sickle+Cell+Anemia+and+umbilical+cord+blood&rank=1)

Publications:
