

HJR 48: Joint Subcommittee Studying Medical, Ethical, and Scientific
Issues Relating to Stem Cell Research
October 17, 2006
House Room C, General Assembly Building

Current Umbilical Cord Blood
Clinical Trials
as of October 13, 2006

Good morning, Mr. Chairman, members of the joint subcommittee. As requested by the chairman, I researched current umbilical cord blood clinical trials. All the information was accumulated on ClinicalTrials.gov, a website run by the National Institutes of Health, which links patients to medical research, so that they may be able to participate in some of the groundbreaking research taking place. I will give a brief background on clinical trials as well as discuss four trials about to be underway in order to inform you all of new developments in research involving cord blood.

Background Info:

- **Clinical Trial** -- A clinical trial is a research study in human volunteers to answer specific health questions; can involve a new drug or treatment
- **Phases** -- there are 4 phases of clinical trials
 - Phase 1 - researchers test an experimental drug or treatment in a small group of people (20-80) for the first time to evaluate safety, determine a safe dosage range, and identify side effects
 - Phase 2 - the experimental study drug or treatment is given to 100-300 to see if it is effective and to further evaluate its safety
 - Phase 3 - the experimental study drug or treatment is given to 1000-3000 to confirm its effectiveness, monitor side effects, compare it to commonly used treatments, and collect information that will allow the experimental drug or treatment to be used safely
 - Phase 4 - post marketing studies delineate additional information including the drug's risks, benefits, and optimal use

- Sponsors of Clinical Trials include: National Institutes of Health, other federal agencies (ex. DOD, Veteran's Affairs), industry, the Clinical Research Network, and University/Organizations
- According to ClinicalTrials.gov (a website run by NIH, linking patients to medical research), as of October 13, 2006 there are currently 65 clinical trials recruiting volunteers for umbilical cord blood research -- of which 37 are being funded by the NIH
- 88 total in the system -- including those that are completed, or are no longer accepting patients
- There are 53 clinical trials currently recruiting volunteers for sickle cell anemia research -- of which 22 are being funded by the NIH
- (596 "stem cell" studies -- of those 21 in Virginia)

Sampling of Trials:

So, what kind of clinical trials am I talking about? On your hand-out I've summarized 4 of the 65 clinical trials involving umbilical cord blood. 3 of them are either fully funded, or partially funded by the NIH. The first one is actually being funded by the University of Florida,

- **Umbilical Cord Blood Infusion to Treat Type 1 Diabetes**
 - Purpose: To transfuse autologous (donor and recipient are the same person) umbilical cord blood into 10 children with T1D in an attempt to regenerate pancreatic islet insulin producing beta cells and improve blood glucose control; and to track the migration of transfused cord blood stem cells and study the potential changes in metabolism/immune function leading to islet regeneration
 - Rationale: As Type 1 Diabetes is still associated with tremendous morbidity and premature mortality, thus the need to find a cure for Type 1 Diabetes cannot be overstated -- autologous stem cell transplants either with their potential to differentiate into islet cells or lead to regeneration appear to be a safe and potentially viable option
 - This study is taking place at the University of Florida
- **Collection and Storage of Umbilical Cord Stem Cells for Treatment of Sickle Cell Disease** - funded by NIH

- Purpose: to 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 cells to change shape and clump together, preventing their normal flow in the bloodstream). Cord blood may prove useful in 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
- The blood will be used in various ways -- (1) if 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 including stem cell transplantation or gene therapy; (2) if 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 currently living or future siblings with sickle cell disease.
- This study will be a multi-state collaboration with Washington metropolitan area hospitals (very new -- created in June 2006)
- **Combination Chemotherapy Followed by Donor Bone Marrow or Umbilical Cord Blood Transplant in Treating Children with Newly Diagnosed Juvenile Myelomonocytic Leukemia** - funded by the National Cancer Institute (a component of NIH)
 - Purpose: Phase II trial to study how well giving combination therapy together with donor bone marrow or umbilical cord blood transplant works in treating children with newly diagnosed juvenile myelomonocytic leukemia (rare, accounts for less than 1% of all childhood leukemias) -- *cancer that originates in a marrow cell that normally functions to form blood cells; infants to 4 years usually, fail to thrive, lethargy, fevers, exaggerated bleeding in the skin, mouth or nose, enlargement of the liver may occur, enlargement of the spleen*
 - Rationale: Giving chemotherapy drugs before a donor bone marrow transplant or an umbilical cord transplant helps stop the growth of cancer cells and helps stop the patient's immune system from rejecting the donor's stem cells. Additionally when the healthy stem cells from a donor are infused into the patient they may help the patient's bone marrow make stem cells, red blood cells, white blood cells, and platelets.
 - Objectives: to determine: (1) the response rate of children with newly diagnosed juvenile myelomonocytic leukemia treated with chemotherapy drugs (R115777, isotretinoin, cytarabine, and fludarabine) followed by

allogeneic (2 people, related or unrelated) bone marrow or umbilical cord blood transplantation; (2) the safety and toxicity of this regimen; (3) the tolerability of this regimen; (4) the rate of 2-year event-free survival of patients treated with this regimen

- multi-center study -- with participating locations in VA -- Children's Hospital of The King's Daughters in Norfolk, INOVA Fairfax Hospital, and VCU Massey Cancer Center here in Richmond.

- **Umbilical Cord Blood and Placental Blood Transplantation in Treating Patients with Hematologic Cancer or Aplastic Anemia** - partially funded by NIH (National Cancer Institute)

- Purpose: Phase II trial to study the effectiveness of umbilical cord blood and placental blood transplantation in treating patients who have hematologic cancer (leukemia, lymphoma -- blood cancers) or aplastic anemia (bone marrow does not produce enough or any new cells to replenish blood cells)
- The rationale for the study is that this type of transplantation may be able to replace immune cells that were destroyed by the chemotherapy or radiation therapy that was used to kill cancer cells
- Objectives include: (1) Determination the response rate of patients with hematologic cancers or aplastic anemia treated with allogeneic (involving two people -- related or unrelated) umbilical cord and placental blood transplantation; (2) Determination of survival in these patients treated with this regimen; (3) Determination of toxicity of this regimen in these patients
- This study is taking place in New York