ClinicalTrials.gov

Safety and Effectiveness of Cord Blood Stem Cell Infusion for the Treatment of Cerebral Palsy in Children

This study is currently recruiting participants.
Verified October 2012 by Georgia Regents University
Sponsor:
Georgia Regents University
Information provided by (Responsible Party):
James E. Carroll, Georgia Health Sciences University
ClinicalTrials.gov Identifier:
NCT01072370
First received: February 16, 2010
Last updated: October 4, 2012
Last verified: October 2012
History of Changes

Purpose

The purpose of this study is to test the safety and effectiveness of a cord blood infusion in children who have motor disability due to cerebral palsy (CP). The subjects will be children whose parents have saved their infant's cord blood, who have non-progressive motor disability, and whose parents intend to have a cord blood infusion.

Condition	Intervention	Phase
Cerebral Palsy	Biological: Cord Blood Infusion Biological: Intravenous Sham	Phase 1 Phase 2

Study Type: Interventional

Study Design: Allocation: Randomized
Endpoint Classification: Safety/Efficacy Study

Intervention Model: Crossover Assignment
Masking: Double Blind (Subject, Caregiver, Investigator, Outcomes Assessor)
Primary Purpose: Treatment

Official Title: A Placebo-Controlled, Observer-Blinded, Crossover Study to Evaluate the Safety and Effectiveness of a Single, Autologous, Cord Blood Stem Cell Infusion for the Treatment of Cerebral Palsy in Children

Resource links provided by NLM:

MedlinePlus related topics: Cerebral Palsy Paralysis

Genetic and Rare Diseases Information Center resources: Cerebral Palsy

U.S. FDA Resources

Further study details as provided by Georgia Regents University:

Primary Outcome Measures:

- Confirm the safety of autologous cord blood infusion in children with cerebral palsy by repeated follow-up over one year with clinical and laboratory evaluations.
  [Time Frame: 1 year] [Designated as safety issue: Yes]

Secondary Outcome Measures:

- Confirm the efficacy of autologous cord blood infusion in children with cerebral palsy using patient questionnaire and standardized Gross Motor Function Measure evaluation.
  [Time Frame: 3-4 months] [Designated as safety issue: No]

Estimated Enrollment: 40

Study Start Date: January 2010

Estimated Study Completion Date: February 2014

Estimated Primary Completion Date: February 2013 (Final data collection date for primary outcome measure)

Arms	Assigned Interventions
Active Comparator: Treatment Group 1	Biological: Cord Blood Infusion red-cell depleted, mononuclear cell enriched cord blood unit

	prepared for infusion Other Name: Stem cell infusion
Sham Comparator: Treatment Group 2	Biological: Intravenous Sham intravenous infusion of 5% dextrose, ¼ normal saline solution Other Name: Placebo

Detailed Description:

The purpose of this study is to conduct an observer-blinded crossover investigation of the safety and efficacy of autologous cord blood infusion in children who demonstrate non-progressive motor disability due to brain dysfunction (commonly called cerebral palsy) and who do not have an apparent disorder of brain development or obstructive hydrocephalus. The degree of delay in motor development will be such that the children are unable to sit independently by 12 months of age or unable to walk independently by 18 months of age. However, because the diagnosis is one of exclusion, we will enroll patients only after they have reached two years of age. By this age, it is likely other conditions would be excluded. As the GMFCS was developed for children up to 12 years of age, the maximum age of recruitment will be 12 years. Any level of cerebral palsy severity will be allowed. The subjects will be children whose parents have saved their infants cord blood, who have clinical evidence of a non-progressive motor disability, and whose parents intend to have a cord blood infusion.

Eligibility

Ages Eligible for Study: 1 Year to 12 Years
Genders Eligible for Study: Both
Accepts Healthy Volunteers: No
Criteria
Inclusion Criteria:

- Must be more than 1 year of age and less than 12 years of age at the time of screening for inclusion in the study.
- Clinical evidence of a non-progressive motor disability due to brain dysfunction. The subjects will not have the ability to sit independently by one year of age or the ability to walk by 18 months of age.
- Have stored umbilical cord blood with CBR that meets all selection and testing criteria.
- Willing to comply with all study procedures.
- The nucleated cells available in the cord blood sample stored at CBR must exceed 1 X 107 cells per kg body weight. (Note: Because cord blood collection has been in process for about 16 years but widely for far less than that period, the age of most subjects likely will be considerably less than 12 years of age. Due to the amount due of cord blood available for most subjects, the body weight of subjects usually will not exceed 25 kg).
- The patients must be seizure-free or seizures adequately controlled. If there is a suspicion of seizures and EEG should be done prior to inclusion.

Exclusion Criteria:

- Have complicating medical issues that would interfere with blood drawing, such as venous access so limited that success is unlikely
- Presence of obstructive hydrocephalus.
- Presence of progressive neurological disease.
- Presence of significant defect of brain development, such as schizencephaly or agenesis of corpus callosum
- Presence of known chromosomal anomaly
- Presence of major congenital anomaly
- Severe intrauterine growth restriction (birth weight less than 1800 grams)
- Cord blood viability <60%
- Positive infectious disease markers from mother's blood or cord blood at the time of collection.
- Evidence of illness on planned infusion date (such as but not limited to fever >38.5, vomiting, diarrhea, wheezing, or crackles)
- Pregnancy
- Use of immunosuppressive drugs
- Evidence of known genetic disorder
- Impaired hepatic or renal function

Contacts and Locations

Please refer to this study by its ClinicalTrials.gov identifier: NCT01072370

Contacts

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Locations

United States, Georgia

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Contact: James E Carroll, M.D. 706-721-3371 jcarroll@georgiahealth.edu
Principal Investigator: James E Carroll, M.D.
Sub-Investigator: Elizabeth Sekul, M.D.
Sub-Investigator: Lloyd O Cook, M.D.
Sub-Investigator: Roni Bollag, M.D., Ph.D.
Sub-Investigator: Suzanne Strickland, MD
Sub-Investigator: Afshin Ameri, MD
Sub-Investigator: Cindy Neunert, MD
Sub-Investigator: Theodore Johnson, MD, PhD

Sponsors and Collaborators

Georgia Regents University

Investigators

Study Chair: James E Carroll, M.D. Georgia Regents University
More Information

Additional Information:
Georgia Health Sciences University
Cord Blood Registry
Georgia Health Sciences University Department of Neurology

No publications provided

Responsible Party:	James E. Carroll, Professor and Chief, Child Neurology, Georgia Health Sciences University
ClinicalTrials.gov Identifier:	NCT01072370 History of Changes
Other Study ID Numbers:	ACBSC09
Study First Received:	February 16, 2010
Last Updated:	October 4, 2012
Health Authority:	United States: Food and Drug Administration

Keywords provided by Georgia Regents University:
Umbilical Cord Blood
Stem Cells
Autologous Blood Transfusion
Cerebral Palsy

Additional relevant MeSH terms:

Cerebral Palsy	Central Nervous System Diseases
Paralysis	Nervous System Diseases
Brain Damage, Chronic	Neurologic Manifestations
Brain Diseases	Signs and Symptoms

ClinicalTrials.gov processed this record on February 27, 2014