A Randomized Study of Autologous Umbilical Cord Blood Reinfusion in Children With Cerebral Palsy

This study is currently recruiting participants.

Verified by Duke University, June 2010
First Received: June 17, 2010 No Changes Posted

Sponsor:	Duke University
Collaborator:	Roberson Foundation (funding)
Information provided by:	Duke University
ClinicalTrials.gov Identifier:	NCT01147653

Purpose

The purpose of this study is to determine the efficacy of a single intravenous infusion of autologous umbilical cord blood (UCB) for the treatment of pediatric patients with spastic cerebral palsy.

Condition	Intervention	Phase
Cerebral Palsy CP Spastic Cerebral Palsy	Biological: Autologous Umbilical Cord Blood or Placebo	Phase II

Study Type:	Interventional
Study Design:	Allocation: Randomized Endpoint Classification: Efficacy Study Intervention Model: Crossover Assignment Masking: Double Blind (Subject, Caregiver, Investigator, Outcomes Assessor) Primary Purpose: Treatment
Official Title:	Is Autologous Umbilical Cord Blood Reinfusion Beneficial in Children With Cerebral Palsy: A Randomized, Blinded, Placebo-Controlled, Crossover Study

Resource links provided by NLM:

MedlinePlus related topics: Cerebral Palsy Paralysis
U.S. FDA Resources

Further study details as provided by Duke University:

Primary Outcome Measures:

- The primary measure of efficacy will be improvement of standardized measures of neurodevelopmental function. [Time Frame: 2 years]
  [Designated as safety issue: No]

Secondary Outcome Measures:

- A secondary objective is to determine effects on quality of life in these children. [Time Frame: 2 years] [Designated as safety issue: No]
- A secondary objective is to describe functional and morphologic changes on brain MRI in these children. [Time Frame: 2 years] [Designated as safety issue: No]
- A secondary objective is to ask whether there is a correlation between clinical response and RNA expression of neural, endotheial and inflammatory cytokines measured by RNA arrays in cord blood cells given to these patients.
  [Time Frame: 2 years] [Designated as safety issue: No]

Estimated Enrollment:	120
Study Start Date:	June 2010
Estimated Study Completion Date:	July 2013
Estimated Primary Completion Date:	July 2012 (Final data collection date for primary outcome measure)

Arms	Assigned Interventions
Autologous Umbilical Cord Blood Reinfusion: Active Comparator All participants will be treated with autologous cord blood reinfusion, but the time course will vary between groups and participants will be blinded to the order in which they receive infusions. Intervention: Biological: Autologous Umbilical Cord Blood or Placebo	Biological: Autologous Umbilical Cord Blood or Placebo All participants will be treated with autologous cord blood reinfusion, but the time course will vary between groups and participants will be blinded to the order in which they receive infusions. Patients will be randomized to receive their autologous umbilical cord blood cells first or placebo first. Subjects will receive both infusions but will be randomized and blinded by which they are receiving first and second.
Placebo: Placebo Comparator All participants will be treated with autologous cord blood reinfusion, but the time course will vary between groups and participants will be blinded to the order in which they receive infusions. Intervention: Biological: Autologous Umbilical Cord Blood or Placebo	Biological: Autologous Umbilical Cord Blood or Placebo All participants will be treated with autologous cord blood reinfusion, but the time course will vary between groups and participants will be blinded to the order in which they receive infusions. Patients will be randomized to receive their autologous umbilical cord blood cells first or placebo first. Subjects will receive both infusions but will be randomized and blinded by which they are receiving first and second.

Detailed Description:

Cerebral palsy results from in utero or perinatal injury to the developing brain, often through stroke, hypoxic insult or hemorrhage. Currently available treatments for patients with cerebral palsy are supportive, but not curative. Umbilical cord blood (UCB) has been shown to lessen the clinical and radiographic impact of hypoxic brain injury and stroke in animal models. UCB also engrafts and differentiates in brain, facilitating neural cell repair, in animal models and human patients with inborn errors of metabolism undergoing allogeneic, unrelated donor UCB transplantation. We hypothesize that, in the setting of brain injury, infusion of autologous UCB will facilitate neural cell repair resulting in improved function in pediatric patients with cerebral palsy.

Eligibility

Ages Eligible for Study: 12 Months to 6 Years
Genders Eligible for Study: Both
Accepts Healthy Volunteers: No
Criteria
Inclusion Criteria:

- Age ≥ 12 months and ≤ 6 years
- Diagnosis: Spastic cerebral palsy with diplegia, hemiplegia, or quadraplegia.
- Performance status: Gross Motor Function Classification Score levels II - IV as determined by the Gross Motor Function Measure-66 (see Appendix 1).
- Autologous umbilical cord blood available at a private or public cord blood bank with a minimum total nucleated cell dose of ≥ 1 x 107 cells/kilogram.
- Parental consent.

Exclusion Criteria:

- Athetoid cerebral palsy.
- Autism and autistic spectrum disorders without motor disability.
- Hypsarrhythmia.
- Intractable seizures causing epileptic encephalopathy.
- Evidence of a progressive neurologic disease.
- Known HIV or uncontrolled bacterial, fungal, or viral infections.
- Impaired renal or liver function as determined by serum creatinine >1.5mg/dL and/or total bilirubin >1.3mg/dL.
- Head circumference >3 standard deviations below the mean for age.
- Known genetic disease or phenotypic evidence of a genetic disease on physical examination.
- Concurrent genetic or acquired disease or comorbidity(ies) that could require a future allogeneic stem cell transplant.
- Requires ventilatory support, including home ventilator, CPAP, BiPAP, or supplemental oxygen.
- Patient's medical condition does not permit safe travel.
- Previously received any form of cellular therapy.
- Autologous umbilical cord blood unit has any of the following:
    1. Total nuclear cell dose < 1 x 107 cells/kilogram
    2. Positive maternal infectious disease markers (except CMV)
    3. Evidence of infectious contamination of the cord blood unit
    4. Lack of a test sample to confirm identity
    5. Evidence of a genetic disease
- Unable to obtain parental consent.

Contacts and Locations

Please refer to this study by its ClinicalTrials.gov identifier: NCT01147653

Contact: Jessica Sun, MD 919-668-1100 jessica.sun@duke.edu

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Locations

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Sponsors and Collaborators

Duke University

Roberson Foundation (funding)

Investigators

Principal Investigator: Joanne Kurtzberg, MD Duke University

More Information

No publications provided

Responsible Party:	Duke University Medical Center (Dr. Joanne Kurtzberg)
ClinicalTrials.gov Identifier:	NCT01147653 History of Changes
Other Study ID Numbers:	eIRB 17801
Study First Received:	June 17, 2010
Last Updated:	June 17, 2010
Health Authority:	United States: Food and Drug Administration; Unites States: Duke University Health Systems Institutional Review Board

Keywords provided by Duke University:

Cerebral Palsy	Cord Blood
CP	Umbilical Cord Blood
Spastic Cerebral Palsy	Autologous Cord Blood

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 January 24, 2011