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

<table>
<thead>
<tr>
<th>Sponsor:</th>
<th>Duke University</th>
</tr>
</thead>
<tbody>
<tr>
<td>Collaborator:</td>
<td>Roberson Foundation (funding)</td>
</tr>
<tr>
<td>Information provided by:</td>
<td>Duke University</td>
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<tr>
<td>ClinicalTrials.gov Identifier:</td>
<td>NCT01147653</td>
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</tbody>
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**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.

<table>
<thead>
<tr>
<th>Condition</th>
<th>Intervention</th>
<th>Phase</th>
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</thead>
<tbody>
<tr>
<td>Cerebral Palsy</td>
<td>Biological: Autologous Umbilical Cord Blood or Placebo</td>
<td>Phase II</td>
</tr>
<tr>
<td>CP</td>
<td></td>
<td></td>
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<tr>
<td>Spastic Cerebral Palsy</td>
<td></td>
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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
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, endothelial 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)
<table>
<thead>
<tr>
<th>Arms</th>
<th>Assigned Interventions</th>
</tr>
</thead>
<tbody>
<tr>
<td>Autologous Umbilical Cord Blood Reinfusion: Active Comparator</td>
<td>Biological: Autologous Umbilical Cord Blood or Placebo</td>
</tr>
<tr>
<td>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</td>
<td></td>
</tr>
<tr>
<td>Placebo: Placebo Comparator</td>
<td>Biological: Autologous Umbilical Cord Blood or Placebo</td>
</tr>
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<td>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</td>
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**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 10^7 cells/kilogram.
- Parental consent.

Exclusion Criteria:

- Athetoid cerebral palsy.
- 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 10^7 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
Contact: June Allison, RN  919-668-1100  allis006@mc.duke.edu

Locations

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Durham, North Carolina, United States, 27705
Contact: Jessica Sun, MD  jessica.sun@duke.edu
Principal Investigator: Joanne Kurtzberg, MD
Sub-Investigator: Jessica Sun, MD

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;  United 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