

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

This study is currently recruiting participants.

[Verified October 2013](#) by Duke University

Sponsor:

Joanne Kurtzberg

Collaborator:

Roberson Foundation (funding)

Information provided by (Responsible Party):

Joanne Kurtzberg, Duke University Medical Center

ClinicalTrials.gov Identifier:

NCT01147653

First received: June 17, 2010

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[History of Changes](#)

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- [No Study Results Posted](#)
- [Disclaimer](#)
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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.

<a href="#">Condition</a>	<a href="#">Intervention</a>	<a href="#">Phase</a>
Cerebral Palsy CP Spastic Cerebral Palsy	Biological: Autologous Umbilical Cord Blood or Placebo	Phase 2

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](#)

[Genetic and Rare Diseases Information Center](#) resources: [Cerebral Palsy](#)

[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, 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: January 2016

Estimated Primary Completion Date: January 2016 (Final data collection date for primary

outcome measure)

<u>Arms</u>	<u>Assigned Interventions</u>
<p>Active Comparator: Autologous Umbilical Cord Blood Reinfusion 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.</p>	<p>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.</p>
<p>Placebo Comparator: 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.</p>	<p>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.</p>

### 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  $\geq$  12 months and  $\leq$  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  $\geq 1 \times 10^7$  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  $>1.5\text{mg/dL}$  and/or total bilirubin  $>1.3\text{mg/dL}$ .
- 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  $< 1 \times 10^7$  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

#### Contacts

Contact: Jessica Sun, MD 919-668-1100 [jessica.sun@duke.edu](mailto:jessica.sun@duke.edu)

Contact: Erin Arnold, RN 919-668-8281 [erin.arnold@dm.duke.edu](mailto:erin.arnold@dm.duke.edu)

## Locations

### United States, North Carolina

Duke University Medical Center                      Recruiting  
Durham, North Carolina, United States, 27705  
Contact: Jessica Sun, MD    [jessica.sun@duke.edu](mailto:jessica.sun@duke.edu)  
Principal Investigator: Joanne Kurtzberg, MD  
Sub-Investigator: Jessica Sun, MD

### Sponsors and Collaborators

Joanne Kurtzberg  
Roberson Foundation (funding)

### Investigators

Principal Investigator: Joanne Kurtzberg, MD    Duke University

▶ [More Information](#)

No publications provided

Responsible Party: Joanne Kurtzberg, Professor of Pediatrics, Duke University Medical Center

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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 February 27, 2014