Safety and Effectiveness of Banked Cord Blood or Bone Morrow Stem Cells in Children With Cerebral Palsy (CP). (ACT for CP)

This study is currently recruiting participants.
Verified January 2014 by The University of Texas Health Science Center, Houston
Sponsor:
The University of Texas Health Science Center, Houston
Collaborators:
Cord Blood Registry (CBR) Inc.
Let's Cure CP Foundation
TIRR Foundation
Information provided by (Responsible Party):
Charles Cox, The University of Texas Health Science Center, Houston
ClinicalTrials.gov Identifier:
NCT01988584
First received: November 4, 2013
Last updated: January 21, 2014
Last verified: January 2014
History of Changes

Purpose

The purpose of this study is to compare the safety and effectiveness of two types of stem cells,(either banked cord blood or bone marrow), in children between the ages of 2 to 10 years with CP. 15 children with banked cord blood at CBR and 15 children without banked cord blood will be enrolled into the study. The study involves one baseline/treatment visit and 3 follow-up visits at 6 months, 12 months, and 2 years. Five children in each group will be randomized to a placebo control group at the baseline/treatment visit. Parents will not be told if their child received stem cells or a placebo until the 12 month follow-up visit. At that time parents may elect to have their child receive the stem cell treatment; either bone morrow harvest or umbilical

cord blood if banked with CBR. All study visits will be conducted at the UTHealth Medical School and Children's Memorial Hermann Hospital in Houston, Texas.

As of 1/21/2014 we have met our enrollment limit for children without banked cord blood undergoing bone marrow harvest for stem cells.

Condition	Intervention	Phase
Cerebral Palsy	Biological: Autologous Stem Cells	Phase 2

Study Type: Interventional

Study Design: Allocation: Randomized
Endpoint Classification: Safety/Efficacy Study
Intervention Model: Crossover Assignment
Masking: Double Blind (Subject, Caregiver, Outcomes Assessor)
Primary Purpose: Treatment

Official Title: Autologous Cell Therapies for Cerebral Palsy-Chronic (ACT for CP)

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 The University of Texas Health Science Center, Houston:

Primary Outcome Measures:

- To determine if autologous cells using either BMMNCs or hUCBs are safe to administer in children with CP by assessing change at multiple time points. [Time Frame: All study visits from baseline to the end of study visit at year 2.] [Designated as safety issue: Yes]
    1. In-hospital infusion toxicity: pulmonary and hepatic function; new seizures, hemorrhagic lesions or ischemic lesions on imaging. (Composite Outcome Measure)
    2. Long-term safety: development of new mass lesions or other pathological structural changes; worsening neurological status. (Composite Outcome Measure)

Secondary Outcome Measures:

- To determine if late functional outcomes are improved following the administration of autologous cells compared with patients in the control group. [Time Frame: Follow-up visits at 6 and 12 months, and the end of study year 2 visit.]
  [Designated as safety issue: No]
  1. Detailed analysis of MRI's done at baseline and follow-up visits. Specific white matter tract analysis will be identified at baseline MRI and correlated with motor function studies as the primary lesion of interest. Total volumes and specific tract lesions will be analyzed and correlated with functional outcomes.
  2. The following functional outcomes studies will be performed at baseline, 6 months, 1 year after infusion, and 2 year after infusion.
     - Gross Motor Function Measures
     - Psychological Assessment Tests

Estimated Enrollment:	30
Study Start Date:	November 2013
Estimated Primary Completion Date:	November 2015 (Final data collection date for primary outcome measure)

Arms	Assigned Interventions
Active Comparator: Umbilical Cord Blood (UCB) Arm Children who have banked UCB with CBR will receive an umbilical cord blood stem cell infusion at the baseline/treatment visit.	Biological: Autologous Stem Cells Other Name: Autolgous stem cells; from either banked umbilical cord blood or bone morrow harvest.
Active Comparator: Bone Marrow Stem Cells (BMMNC's) Children in the BMMNC group will undergo bone marrow harvest and stem cell infusion at the baseline/treatment visit.	Biological: Autologous Stem Cells Other Name: Autolgous stem cells; from either banked umbilical cord blood or bone morrow harvest.
Placebo Comparator: Placebo (inactive substance) Group Five children in each group will be randomly assigned to receive an inactive substance (placebo) at the baseline/treatment visit. Parents will be given the opportunity to cross-over to either the umbilical cord blood or bone marrow harvest group at the one year visit.	

CURE CP

Eligibility

Ages Eligible for Study:	2 Years to 10 Years
Genders Eligible for Study:	Both
Accepts Healthy Volunteers:	No

Criteria

Inclusion Criteria:

1. Children with diagnosis of Cerebral Palsy (spastic CP due to periventricular white matter damage or neonatal brain injury from perinatal stroke or intra-ventricular hemorrhage)
2. Gross Motor Function Classification Score level II-V
3. Ages 24 months to 10 years
4. English speaking, if verbal
5. Ability to travel to Houston for treatment and follow-up -

Exclusion Criteria:

1. Known history of:
    - Intractable seizures
    - Traumatic brain injury
    - Genetic disorder (as demonstrated by newborn screening or genetic diagnostic testing)
    - Recently treated or current infection
    - Renal insufficiency or altered renal function (as defined by serum creatinine > 1.5 mg/dl at screening)
    - Hepatic disease or altered liver function (as defined by SGPT > 150 U/L [non-contusion related], and/or T. Bilirubin >1.3 mg/dL at screening)
    - HIV+ (as demonstrated by positive blood test)
    - Immunosuppression (as defined by WBC <3,000 cells/ml at screening)
    - Infectious related neurological injury
    - Sensitivity to Ethylene Oxide (EtO) [found in fumigants and disinfectants]
2. If Athetoid CP diagnosis, other etiologies such as degenerative, mitochondrial, and metabolic disorders must be excluded, and the outcome assessments must be able to be conducted to assess for potential treatment effects
3. Normal brain MRI
4. Evidence of acute illness at the time of infusion, such as, but not limited to, fever (temperature > 37.5 C), vomiting, diarrhea, wheezing or crackles
5. Progressing neurological disease (as defined by Batten Disease, Leukodystrophies, Metabolic disorders, Mitochondrial disorders, Neurotransmitter disorders)

6. Microcephaly, macrocephaly, cortical malformations, genetic disorders of dysgenesis brain malformations due to infection or metabolic disorders
7. Pulmonary disease requiring ventilator support
8. If hUCB candidate, banked cord cells totaling <10 million/kg
9. If hUCB candidate, any positive maternal infectious disease test (Hepatitis A, Hepatitis B, HIV 1, HIV 2, HTLV 1, HTLV 2, and Syphilis)
10. If hUCB candidate, cord blood sample contamination
11. Participation in a concurrent intervention study
12. Unwillingness to return for follow-up visits
13. Contraindications to MRI
14. Any patient that the investigators feel in their opinion the study intervention is unlikely to benefit the patient will be a screen failure.
15. Any patients who are currently or has previously been enrolled in a clinical stem cell study.

Contacts and Locations

Please refer to this study by its ClinicalTrials.gov identifier: NCT01988584

Contacts

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Locations

United States, Texas

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Principal Investigator: Charles S Cox, MD

Sponsors and Collaborators

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TIRR Foundation

Investigators

Principal Investigator:	Charles S Cox, MD	UTHealth, Medical School, Dept. of Pediatric Surgery

More Information

No publications provided

Responsible Party:	Charles Cox, The Children's Fund Distinguished Professor, Department of Pediatric Surgery, The University of Texas Health Science Center, Houston
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Keywords provided by The University of Texas Health Science Center, Houston:

Cerebral Palsy	Mononuclear Cells
Brain Injury	Bone Morrow
Stem Cells	Umbilical 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