WHAT IS BRAIN INJURY?

Brain injury in children with sickle cell anemia includes, stroke (brain attack), silent stroke, and blood flow to the brain that is too fast, as tested by transcranial Doppler ultrasound (TCD). Without treatment, 1 in 10 children with sickle cell anemia will have a stroke by age 18 and another 1 in 3 will have a silent stroke. Strokes and silent strokes often reduce intelligence and can lead to difficulty with school work. About 1 in 10 children with sickle cell anemia have blood flow to the brain that is too fast and need regular transfusions to prevent a stroke.

WHAT IS HU PREVENT?

HU Prevent is a study that aims to compare the drug hydroxyurea to a placebo (a liquid that looks the same but contains no active medicine) in children with sickle cell anemia to determine if hydroxyurea can prevent brain injury. Some of the most devastating complications of sickle cell anemia, including stroke, silent stroke, and fast blood flow to the brain, can occur early in a child's life. Through information gained from the HU Prevent Study, we hope to help prevent these common problems in children with sickle cell anemia.

BE A PART OF SOMETHING BIG!

Hospitals across the country are participating in this study in an effort to improve the lives of children and families living with sickle cell anemia. These institutions include:

- Johns Hopkins Children's Center
- Washington University in St. Louis
- University of Alabama at Birmingham
- Vanderbilt University
- Children's Hospital of Philadelphia
- Cincinnati Children's Hospital

Your participation will help us determine how to best treat children with sickle cell anemia in the future.

HU Prevent Clinical Coordinating Center

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JOHNS HOPKINS CHILDREN'S CENTER

Funded by the United States National Institute of Health (NIH)/The National Heart, Lung, and Blood Institute (NHLBI)

Dr. James Casella
Principal Investigator

HU PREVENT

HYDROXYUREA FOR THE PREVENTION OF BRAIN INJURY IN SICKLE CELL DISEASE

Improving the Lives of Children with Sickle Cell Anemia

Study Number: NA_00041623

JOHNS HOPKINS CHILDREN'S CENTER

FREQUENTLY ASKED QUESTIONS

Who can Join the Study?

Any family with a child between the ages of 9 months to 48 months with sickle cell anemia (hemoglobin SS) or sickle beta-null thalassemia.

What Will the Study Involve?

- 2 or 3 visits to see if your child meets the requirements for the study
- History and physical exam with a hematologist and neurologist
- Transcranial Doppler ultrasound (TCD) to measure blood flow to the brain
- Cognitive (intelligence) testing
- MRI of the brain with sedation

Children without brain injury are then randomized (like flipping a coin) to take either liquid hydroxyurea or placebo daily for three years. During that three year period, your child will return for visits every 4 weeks (2 weeks after an increase of dose) for a check-up and blood tests. They will have yearly tests, including a TCD and a MRI, along with neurological and cognitive tests. You will also be eligible for compensation, to help with the costs of visits. Ask your hematologist for more information.

How Long Will the Study Last?

Your & your child's participation in the study will last for 3 years.

Who Else is Involved in this Study?

Other sickle cell patients & their families at Johns Hopkins and at other institutions across the country (see back panel for a full list).

HYDROXYUREA

Hydroxyurea is a drug that increases the amount of fetal (or baby) hemoglobin levels in the blood. Hemoglobin is a protein in red blood cells that carries oxygen. When a person has sickle cell anemia they make sickle hemoglobin. Sickle hemoglobin clumps up when the oxygen in the blood is low and causes the cells to form the sickle shape. These sickle cells can clog up blood vessels and cause brain injury or other sickle cell problems like pain. Fetal hemoglobin helps to stop this clumping of the sickle hemoglobin and is why very young babies with sickle cell anemia rarely have pain or other problems from sickle cell anemia.

As the parent of a child with sickle cell anemia, you desire to give your child the best possible start in life. The first few years of your child's life are important in preparing him or her for a healthy future. By participating in clinical trials such as HU Prevent, patients and health care workers can partner to try to improve the care of patients with sickle cell anemia.