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Study targets stroke prevention in children with sickle cell anemia

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St. Jude Children's Research Hospital investigators were recently awarded a $23 million federal grant to launch a national study of the drug hydroxyurea to prevent first strokes in children and adolescents with sickle cell anemia (SCA).

The effort will be the fifth at St. Jude involving hydroxyurea to treat children with SCA. The focus will be on SCA patients who have not suffered strokes but have been identified as high risk for the complication by transcranial Doppler ultrasound (TCD) screening. The five-year study is expected to include a total of 26 medical centers and about 140 patients.

"Our goal with this study is to prevent brain complications in children with sickle cell disease," explained Russell Ware, M.D., Ph.D., principal investigator for the study, which is funded by the National Heart, Lung, and Blood Institute. Ware chairs the St. Jude Department of Hematology and holds the Lemuel Diggs Endowed Chair in Sickle Cell Disease.

SCA is an inherited blood disorder. In the U.S., it is diagnosed most frequently in African American individuals. The sickled red blood cells they produce can disrupt circulation and cause pain, strokes and other debilitating and sometimes deadly complications.

The new study is called TWiTCH, short for TCD With Transfusions Changing to Hydroxyurea. Patients will be randomly assigned to either continue a standard therapy of monthly blood transfusions and chelation

to remove the resulting iron buildup or to receive a daily dose of hydroxyurea and monthly phlebotomy to remove excess iron. The goal is to compare the two treatments, including their ability to prevent a first stroke.

Source: St. Jude Children's Research Hospital