UT SOUTHWESTERN MEDICAL CENTER NEWS

Media Contact: Kristen Holland Shear
214-648-3404
kristen.hollandshear@utsouthwestern.edu

EMBARGOED UNTIL 5:30 P.M. CDT THURSDAY, MAY 12, 2011

Existing drug treatment reduces pain in young sickle cell anemia patients, UT Southwestern researcher finds in national study

DALLAS – May 12, 2011 – A cancer drug already used to treat adults and school-age children with sickle cell anemia is safe and significantly reduces pain and other complications of the disease in children as young as 9 months, according to a national study involving a UT Southwestern Medical Center researcher.

Pediatric researchers at UT Southwestern and 13 other academic medical centers say hydroxyurea should be offered to all young children with sickle cell anemia, regardless of disease severity and clinical symptoms. The findings of the Pediatric Hydroxyurea in Sickle Cell Anemia, or BABY HUG, trial appear online and in the May 14 edition of the *Lancet*.

"We've offered hydroxyurea at Children's since 1992 to severely involved patients with frequent or severe complication down to age 3. On the basis of the BABY HUG study's findings, our sickle cell team has made a conscious decision to now offer hydroxyurea to all sickle cell anemia patients in the first year of life," said study co-author Dr. Zora Rogers, professor of pediatrics at UT Southwestern and clinical director of the general hematology and bone marrow failure program at Children's Medical Center Dallas.

The findings, Dr. Rogers said, likely will change how all medical professionals treat very young children with sickle cell anemia.

"This medication reduces painful events, the major crisis patients fear about sickle cell disease, as well as the problems doctors fear, which include chest syndrome – a unique complication of pulmonary infarction and infection that only occurs in sickle cell disease – hospitalization and transfusions," she said. "The study also showed a trend of reducing organ damage in the spleen, but the study sample was too small to prove protection."

Sickle cell anemia is an inherited genetic blood disorder in which the bone marrow produces mutant, inflexible, sickle-shaped red blood cells. These cells may aggregate and block small blood vessels within the body, causing pain, organ damage, stroke and premature death. Approximately 100,000 Americans suffer from the disease.

Babies born with sickle cell disease are protected for about six months by fetal hemoglobin. As

(MORE)

fetal hemoglobin levels drop, however, the disease starts its damaging effects. Although hydroxyurea is effective at raising fetal hemoglobin and thus reducing painful events and other crises in adults and older children, researchers were uncertain until now whether the drug could also help babies.

In BABY HUG, researchers wanted to determine whether hydroxyurea therapy would prevent early organ damage in very young children with sickle cell anemia.

From October 2003 to September 2007, researchers enrolled 193 children between the ages of 9 and 19 months and randomly assigned each to receive hydroxyurea or the placebo for two years. A total of 167 (including 12 at Children's) completed the trial – the first randomized double-blind trial to examine the drug's effect in very young children with sickle cell anemia.

"We found a decrease in chest syndrome and hospitalization among trial participants who received hydroxyurea," said Dr. Rogers, adding that the findings represent the culmination of 15 years of work at UT Southwestern and Children's. "We used to offer hydroxyurea as secondary prevention, but with these findings, it could become the primary preventive measure."

She stressed that the drug is only effective as a preventive measure. "This is not a therapy when a crisis occurs," Dr. Rogers said. "Patients may still experience painful crises, but the events are much less frequent and severe."

The next step, Dr. Rogers said, is to make available a standardized liquid form of the drug. The results of BABY HUG may be used to support a Food and Drug Administration application of such a new preparation.

In addition to UT Southwestern's participation, researchers from St. Jude's Children's Research Hospital; SUNY Downstate Medical Center; University of Mississippi Medical Center; Johns Hopkins University School of Medicine; Children's National Medical Center; Howard University College of Medicine; Duke University Medical Center; Medical University of South Carolina; University of Miami; Emory University School of Medicine; Children's Hospital of Michigan; University of Alabama at Birmingham; Medical College of Georgia; the National Heart, Lung and Blood Institute; and the Clinical Trials & Surveys Consortium contributed to the study.

The National Heart, Lung and Blood Institute, and the National Institute of Child Health and Human Development supported the study.

###