

From the University of Mississippi Medical Center Division of Public Affairs

UMMC PART OF STUDY FINDING SICKLE CELL TREATMENT SAFE FOR YOUNG CHILDREN

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JACKSON, Miss. – A drug now used to treat adults who have sickle cell disease appears to be safe for children aged eight to 19 months, results from a new National Institutes of Health-funded study suggest. The drug, hydroxyurea, reduced pain episodes and improved key blood measurements in the children studied, according to researchers.

Findings were published in the May 14 issue of *The Lancet*, a lead British medical journal.

The University of Mississippi Medical Center is one of 14 contributing centers in the ongoing study and enrolled a majority of the 193 participants.

Dr. Rathi Iyer, professor of pediatrics at UMMC and a co-author of the multicenter study, wants the sickle cell community to know that this drug is available for prevention and treatment of symptoms or complications. "At present, this is the only drug that helps ameliorate the symptoms of the disease and helps delay organ damage, which starts very early in life," said Iyer.

"There are now strong reasons for health-care professionals to consider starting children who have sickle cell disease as early as possible on hydroxyurea," said Dr. Susan B. Shurin, acting director of the National Heart, Lung, and Blood Institute (NHLBI) of the NIH, which supported the study. "Less pain and fewer hospital stays are obvious improvements in the quality of life for the youngest children born with this condition."

The study, The Pediatric Hydroxyurea Phase III Clinical Trial, known as Baby HUG, was designed to determine whether hydroxyurea could protect spleen and kidney function in very young children who have sickle cell disease. Loss of spleen function is associated with increased risks of serious bacterial infections. The study also sought to determine whether hydroxyurea treatment would reduce the frequency of other complications, including pain events and hospital stays. It is the largest trial to test hydroxyurea treatment in very young patients.

Sickle cell disease is an inherited blood disorder that affects approximately 100,000 Americans. It is most prevalent in persons of African, Hispanic, Mediterranean, and Middle Eastern descent. People living with this disease have two copies of an altered gene responsible for producing hemoglobin, the protein in red blood cells that transports oxygen throughout the body. Those with sickle cell disease produce hemoglobin that changes shape and becomes stiff after releasing its oxygen. The transformation, which can cause normally round and flexible red blood cells to become misshapen and sticky, slows the flow of blood to the tissues.

These changes contribute to fatigue and pain, which are among the hallmarks of this disease. There is no widely available cure for sickle cell disease, though bone marrow transplants have cured some younger patients. Those who live with the disease have life-long anemia due to the rapid destruction of red blood cells in the body. Some people with sickle cell disease undergo periodic blood transfusions to increase the number of healthy red blood cells.

Hydroxyurea was originally developed as a cancer treatment, but has been successful in reducing episodes of severe pain, known as pain crises, in adults with sickle cell disease. The drug is intended to raise levels of fetal hemoglobin, a form of hemoglobin everyone produces before birth and during the first few months of life. As time goes on, this fetal hemoglobin almost disappears from a person's system as

production of adult hemoglobin takes over. Increasing fetal hemoglobin levels for people who have sickle cell disease is helpful because fetal hemoglobin reduces the tendency of sickle hemoglobin to change the shape of the red blood cells.

To arrange an interview with an NHLBI spokesperson, please contact the NHLBI Communications Office at (301) 496-4236 or nhlbi_news@nhlbi.nih.gov.

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