MedicalXpress

Sickle cell anemia drug safe and effective for infants and toddlers, adds treatment option

May 12 2011

New research shows a drug commonly used to treat sickle cell anemia in adults reduces bouts of acute pain and a pneumonia-like illness, cuts hospitalization time and eases other symptoms of the disease in young patients. Results of the randomized, double-blind trial mark a dramatic advance in treatment of children with the inherited blood disorder.

"These results show that hydroxyurea has the potential to dramatically improve the quality of life for an entire generation of patients with sickle cell disease," said Winfred Wang, M.D., the study's principal investigator and a member of the St. Jude Children's Research Hospital Department of Hematology. He said the findings mean hydroxyurea should now be considered for treatment of all infant and toddlers with sickle cell anemia in hopes of preventing or delaying disease complications. The research will be published in the May 14 issue of the British medical journal The Lancet.

St. Jude researchers led the six-year Pediatric Hydroxyurea Phase III Clinical Trial, known as Baby HUG. The federally funded study involved 193 infants and toddlers enrolled at 13 participating U.S. medical centers and a coordinating center.

About 100,000 Americans have sickle cell anemia, a chronic disorder associated with a range of health problems, including an increased risk of strokes and premature death. Sickle cell anemia is the most common genetic disorder affecting Americans of African descent, but the disease also strikes persons of other racial and ethnic backgrounds.

Sickle cell anemia is caused by a gene mutation that leaves the red blood cells of sickle cell patients prone to assuming the stiff, crescent shape for which the disease is named. The misshapen cells can clog blood vessels, triggering pain crises, strokes and organ damage, including kidney failure. The drug works in part by increasing production of fetal hemoglobin, which counteracts the effects of the sickle hemoglobin. Fetal hemoglobin is the main hemoglobin produced by all newborns, but production normally decreases dramatically within a few months after birth.

While life expectancy for sickle cell anemia patients has improved in recent decades thanks in part to better supportive care, Wang said hydroxyurea is the first drug proven to reduce the incidence of a wide range of symptoms in extremely young sickle cell patients regardless of disease severity. The drug is inexpensive and easy to administer. The drug has been used for more than 15 years as a treatment for sickle cell disease with no evidence of serious side effects. Hydroxyurea began as a potential cancer treatment, but won U.S. Food and Drug Administration approval for use in adults with severe sickle cell disease. Baby HUG is the largest trial of hydroxyurea in much younger patients.

The Baby HUG trial was launched in 2003 after promising preliminary results regarding the drug's safety and effectiveness in extremely young children. The research included patients who were ages 9 to 18 months when they began the study. Participants were randomly assigned to receive either a standard dose of hydroxyurea or a placebo every day for two years. Neither the families nor the caregivers knew which children received hydroxyurea.

An analysis of 179 patients who completed at least 18 months of the study found children in the placebo group had nearly twice as many acute pain episodes, were three times more likely to suffer a pneumonia-like illness known as acute chest syndrome and five times more likely to

develop painful swelling of the hands and feet called dactylitis. They were also slightly more likely to be hospitalized or need blood transfusions to ease sickle cell symptoms.

The most common side effect reported in this study was a mild-to-moderate drop in the white blood cells known as neutrophils, which occurred more often in children receiving hydroxyurea. Low neutrophil counts can be associated with an increased risk of infection, but there was no evidence of this in the Baby HUG trial.

Although results of kidney and spleen function tests were not significantly different between the Baby HUG treatment groups, Wang said other measures suggested that hydroxyurea might protect those organs as well as the brain and lungs from the chronic damage that leaves sickle cell anemia patients at increased risk for premature death. A follow-up study to Baby HUG is underway, focusing on possible long-term benefits from continued treatment with higher doses of hydroxyurea.

Provided by St. Jude Children's Research Hospital

Citation: Sickle cell anemia drug safe and effective for infants and toddlers, adds treatment option (2011, May 12) retrieved 5 May 2024 from https://medicalxpress.com/news/2011-05-sickle-cell-anemia-drug-safe.html

This document is subject to copyright. Apart from any fair dealing for the purpose of private study or research, no part may be reproduced without the written permission. The content is provided for information purposes only.