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# Drug treatment means better, less costly care for children with sickle cell disease

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The benefits of hydroxyurea treatment in people with sickle cell disease are well known—fewer painful episodes, fewer blood transfusions and fewer hospitalizations. Now new research from the Johns Hopkins Children's Center and other institutions reveals that by preventing such complications, the drug can also considerably lower the overall cost of medical care in children with this condition.

The cost-benefit analysis, described online Sept. 2 in the journal *Pediatrics* and believed to be the first of its kind in pediatric patients, showed that children whose standard care was augmented with daily hydroxyurea incurred, on average, $3,000 less in medical costs per year, compared with children who got standard therapy plus placebo.

"In addition to alleviating the human suffering in sickle cell disease, we found that hydroxyurea had the pleasant 'side effect' of substantially lowering the cost of care among children treated with it," says study investigator James Casella, M.D., director of pediatric hematology at the Johns Hopkins Children's Center. "Our main goal is to find better treatments, but in a time when we are trying to curb health care spending while improving patient outcomes, this is really welcome news."

Although proven valuable in therapy of sickle-cell disease, hydroxyurea remains woefully underused in the treatment of people with this condition, the investigators say. The new findings, the research team says, should encourage more physicians to consider adding hydroxyurea to their patients' therapeutic regimens.

"Our analysis specifically demonstrates an added benefit to the use of hydroxyurea for treatment of sickle cell disease related to the drug's impact on the cost of medical care and should be another factor in support of more widespread use of the drug in both children and adults," says the study's lead investigator Winfred Wang, M.D., of the Hematology Department at St. Jude Children's Research Hospital.

Sickle cell disease, a genetic disorder that affects people of African, Middle Eastern, Mediterranean and Hispanic descent, is marked by abnormally C-shaped or "sickled" red blood cells—the cells that store and distribute oxygen throughout the body. Because of their abnormal shape, the cells periodically clump inside blood vessels, blocking circulation, which can lead to severe anemia, drive up infection and stroke risk and cause episodes of extreme pain.

Hydroxyurea—the only FDA-approved drug to treat the excruciating pain crises of sickle cell disease—works by boosting the levels of a type of hemoglobin that makes red blood cells less likely to bend and clump. Although the drug's beneficial effects on alleviating sickle cell pain were first demonstrated at Johns Hopkins in the mid-1990s, the compound was first synthesized in the late 1800s and has been used mainly in the treatment of certain blood cancers.

In the current study, investigators reviewed the number and cost of hospitalizations among 167 sickle cell patients, ages 1 to 3, treated at three pediatric hospitals between 2003 and 2009. Half of the patients received hydroxyurea in addition to their standard care of pain medication and periodic blood transfusions, while the remainder received standard care plus placebo.

Children treated with hydroxyurea were hospitalized 232 times, compared with 324 hospitalizations among those treated without it. The average cost of hospitalization for children who received hydroxyurea

was $9,450 compared with $13,716 among those who received placebo instead. Outpatient treatment was more expensive among children who received hydroxyurea ($1,622 per year), compared with those treated without it ($246), but the higher outpatient cost was offset by fewer hospitalizations and fewer complications. The average cost of all care, including inpatient and outpatient, among children treated with hydroxyurea was $11,072, compared with $13,962 for those treated with placebo.

The lower cost associated with hydroxyurea therapy stems from the compound's ability to prevent or reduce the occurrence of pain crises and serious respiratory complications that require blood transfusions and other treatments, the researchers say. An earlier study by the same group found that children treated with hydroxyurea had fewer such complications than children not receiving the treatment.

About 100,000 people have sickle cell disease in the United States.

Provided by Johns Hopkins University School of Medicine