MedicalXpress

New study demonstrates how bone marrow transplant can cure sickle cell disease

November 7 2008

A unique approach to bone marrow transplantation pioneered in part by a Children's Hospital of Pittsburgh of UPMC physician has proven to be the only safe and effective cure for sickle cell disease, according to a new study.

Lakshmanan Krishnamurti, MD, a pediatric hematologist/oncologist at Children's Hospital, helped pioneer a form of bone marrow transplantation which relies on reduced-intensity conditioning (RIC). RIC regimens are less toxic to patients and therefore can be offered to patients with severe sickle cell disease because they eliminate life-threatening side effects generally associated with bone marrow transplantation.

In a study published in the November issue of the journal *Biology of Blood and Marrow Transplantation*, Dr. Krishnamurti and colleagues report that six of seven sickle cell patients who received RIC bone marrow transplants in the last decade now have donor marrow and are free from symptoms of their sickle cell disease. Dr. Krishnamurti led five of the seven transplants in the study.

"Bone marrow transplant is the only known cure for sickle cell disease. But doctors have avoided performing them in these patients because complications from a traditional bone marrow transplant can be life-threatening," said Dr. Krishnamurti, director of the Sickle Cell Program at Children's Hospital. "Through the reduced-intensity approach we developed, the potential for complications is dramatically lessened. This

study offers hope for a cure to thousands of patients with severe sickle cell disease."

Traditionally, bone marrow transplants require heavy doses of chemotherapy prior to transplant in order to destroy the recipient's bone marrow so it will not reject the donated marrow. But with their bone marrow destroyed, transplant recipients become vulnerable to life-threatening complications, a risk viewed as unnecessary because sickle cell disease is not typically immediately life-threatening.

Dr. Krishnamurti was the first physician in the world to perform a reduced-intensity bone marrow transplant in a patient with sickle cell disease while at the University of Minnesota in 1999. He joined Children's Hospital in 2003 and that year, he performed the region's first successful bone marrow transplant in a patient with sickle cell disease. Austin Jones, then 5, of Indiana, Pa., underwent an RIC bone marrow transplant Aug. 8, 2003, with donor marrow from his brother, Anthony Jr. Today, Austin, now 10, is free of sickle cell disease.

Source: Children's Hospital of Pittsburgh