SOUTHWESTERN NEWS

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BONE MARROW TRANSPLANT CURES SICKLE CELL ANEMIA IN CHILDREN

DALLAS - August 12, 1996 - Bone marrow transplantation is 70 to 90 percent effective in curing severely affected children with sickle cell anemia, an international four-year trial has concluded.

UT Southwestern Medical Center at Dallas was one of more than a dozen sites worldwide investigating the risks and benefits of bone marrow transplantation for sickle cell disease since 1991. One child was transplanted at UT Southwestern in 1995; however, he was one of four patients in which the transplant was unsuccessful.

"This is a landmark study of bone marrow transplantation for sickle cell disease, and now we know that it is a feasible treatment. Seventy-five percent of the time it works; in fact, it cures the disease," said Dr. George R. Buchanan, professor and director of pediatric oncology, and holder of the Children's Cancer Fund Distinguished Chair in Pediatric Oncology and Hematology.

The results were published in a recent issue of The New England Journal of Medicine. The multi-center collaborative investigation was led by the Fred Hutchinson Cancer Research Center in Seattle, Wash., and included researchers in the United Kingdom, Germany, France and several major American cities.

Sickle cell disease is a genetic blood disorder most common in people of African, Mediterranean and Middle Eastern descent. About 1 in every 400 African-American infants is born with a form of sickle cell disease.

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It is characterized by abnormal hemoglobin, in which the normally round red blood cells are distorted into half-moon or sickle shapes. The abnormal cells clog blood vessels, cutting off oxygen to organs and tissues. This "sickling" can affect every organ in the body.

In the trial, 22 children, ranging in age from 3-years-old to 14-years-old, were enrolled in the trial. Each had a history of severe complications, such as stroke, or a pneumonia-like condition called chest syndrome, or recurrent episodes of acute pain, termed sickle cell crisis. These events contribute to early mortality in patients with sickle cell disease.

Many patients in the trial had previously been treated with repeated blood transfusions to avoid further strokes, crises or organ damage. Some patients with these severe symptoms were excluded from transplantation because of the amount of organ damage they had already sustained. Such symptoms may indicate the need for early transplantation in high risk patients, the authors noted.

To prepare for bone marrow transplant, the patient receives a week of chemotherapy to destroy his own bone marrow. During surgery, marrow is extracted from the hip of a healthy sibling with matching marrow and infused into the sickle-cell patient. If the healthy marrow grows, the disease will be cured because no sickle cells remain, and the recipient will produce normal blood cells.

After a median follow-up of two years, 16 of the 22 patients were cured of the disease. Two patients died – one shortly following the transplant; the other a year later from graft vs. host disease, in which the donor's cells attack the recipient's body.

Three patients experienced rejection of the donor marrow followed by the return of sickle cell disease, and a fourth experienced rejection accompanied by marrow aplasia.

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"What's important to note is that even when the graft was rejected, these four patients went back to where they were before the transplant," said Dr. Zora R. Rogers, assistant professor of pediatrics and associate medical director of the sickle cell clinic at Children's Medical Center of Dallas.

The 90 percent survival and 73 percent event-free survival rates of the patients in this trial mirror a Belgian study that produced 89 percent event-free survival.

"Bone marrow transplantation is only an option for a very few patients with sickle cell anemia," said Rogers. "Only about 1 percent or 2 percent of all sickle cell patients have the disease severely enough to require transplantation and have a sibling who matches."

Rogers performed the Texas transplant with her colleague Dr. Eric Sandler, assistant professor of pediatrics and director of UT Southwestern's pediatric bone marrow transplant program.

Encouraged by these results in sickle pediatric sickle cell patients, the group of national and international investigators have recently opened a new trial to evaluate the transplant in carefully selected adults with sickle cell anemia.

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