MedicalXpress

Cord blood viable option for kids with life-threatening metabolic disorders

December 10 2007

Children born with inherited metabolic disorders that cause organ failure and early death can be treated successfully with umbilical cord blood transplants from unrelated donors and, in some cases go on to live for many years, according to a study led by Duke University Medical Center researchers.

Umbilical cord blood transplant may confer advantages over bone marrow transplant, which has been the traditional method for treating these disorders, the researchers said.

"During the past 25 years, children with these disorders, which include Hurler disease and Krabbe leukodystrophy, have been treated successfully with bone marrow transplants but only if a matched donor was available," said Vinod Prasad, M.D., a pediatric hematologist/oncologist at Duke and lead investigator on the study. "Umbilical cord blood transplant can be done successfully from a mismatched donor, so it opens the possibility of treatment to many patients who otherwise would succumb to their disorders."

The researchers presented their findings on Dec. 10 at the American Society of Hematology meeting in Atlanta. The study was funded by the National Institutes of Health and Hunter's Hope Foundation, an organization founded in memory of former NFL quarterback Jim Kelly's son Hunter, who died from Krabbe disease, an inherited metabolic disorder that affects the nervous system.

"These disorders are rare when taken individually -- some of them occur in only one in a million births -- but if you put them together they have a sizeable incidence, maybe 1 in 10,000 births," Prasad said. "What these patients have in common is that they have some type of gene defect that causes them to lack a critical enzyme, required for the development of a vital organ, such as the heart or the brain or the nerves."

Without successful intervention, many of these children die before their first birthday, he said. Bone marrow and umbilical cord blood transplant work in these patients in much the same way -- by replacing missing enzymes and allowing the affected organs to develop more normally.

For this study, researchers looked at 159 children with inherited metabolic disorders who received unrelated cord blood transplants at Duke between 1995 and 2007.

"We saw that there were advantages to the unrelated cord blood transplant," Prasad said. "For instance, cord blood is more readily available than bone marrow and there was a decreased risk of complications, including a lower incidence of serious and potentially fatal graft-versus-host disease, which occurs when donor cells perceive a recipient's tissues and organs as foreign."

The study also suggests that when patients are transplanted while they are still relatively healthy, they have better outcomes than their counterparts who received bone marrow transplants.

"Over 88 percent of this subset of patients were alive one year after their cord blood transplants, and close to 80 percent were alive five years afterwards," Prasad said. "One reason for this could be the cord blood cells are immunologically more naïve than the blood-forming stem cells derived from bone marrow and therefore they may be more adaptable and less reactive once they get into the patient's body."

In a previous study looking at bone marrow transplant as a treatment for Hurler disease, which causes damage to the heart, liver and brain, only 35 percent of patients were alive five years after treatment, whereas 58 percent of all patients examined as part of the current study -- those with both high and lower functional status -- were alive after five years, Prasad said.

"Patients with inherited metabolic disorders who could benefit from transplantation should be referred early and diagnosis should be made early by enzyme testing, whenever possible," he said. "If we see them early enough they can have excellent short-term and hopefully long-term outcomes."

Duke has the largest cord blood transplant program in the country, and the first unrelated cord blood transplant was performed by Duke doctor Joanne Kurtzberg in 1993 on a patient with leukemia.

Source: Duke University Medical Center