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Expanded cord blood shows potential for use in adult bone marrow transplants

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Umbilical cord blood stem cells that are cultured and expanded outside the body before being used for bone marrow transplant in adult blood cancer patients appear safe and restore blood count recovery faster than standard cord blood.

The findings, led by a Duke Cancer Institute researcher, are from a phase I/II study of the biologic treatment, NiCord, at 11 clinical trial sites. The study is publishing online Dec. 4 in the *Journal of Clinical Oncology* and advances efforts to improve cord blood use among adults who have been diagnosed with blood cancers.

Cord blood has been shown to be a rich source of stem cells and is commonly used for transplantation in children, but the number of stem cells tends to be too low for adults, discouraging their use. In the expansion process of the investigational therapy, umbilical cord blood undergoes a three-week cultivation of the stem cells outside the patient before transplantation.

"Although umbilical cord blood transplantation has been used for 30 years, expansion technology represents an opportunity to improve the results for adult patients," said Mitchell Horwitz, M.D., professor of medicine at Duke and lead author of the study.

"This study shows that a single unit of this product appears to be delivered safely to patients around the world."

A larger, phase III trial of NiCord is already underway and if results are replicated, the product's manufacturer, Gamida Cell, plans to seek FDA approval of NiCord, Horwitz said. The company provided funding support for the clinical trials.

In their study, Horwitz and colleagues analyzed results for 36 adult patients with blood cancers who received a cord blood transplant with NiCord. This group was compared to a historic group of closely matched patients listed in the Center for International Blood and Marrow Transplant Research databank who received cord blood without expansion.

The researchers reported that 94 percent of patients who received the investigational product had successful engraftment within six weeks. Two patients experienced secondary graft failure attributable to viral infections.

Blood count recovery occurred much earlier among the patients who received the investigational product—an important milestone that improves a patient's ability to fight infections. The median time to neutrophil recovery was 11.5 days for patients receiving the NiCord, compared to 21 days for those receiving cord blood alone. Platelet recovery was 34 days for the NiCord group versus 46 days for cord blood alone.

"Compared to standard cord blood transplant, the reduction in recovery time translates into significant improvement in the safety profile of the transplant procedure," Horwitz said. "It's when their blood counts are low that patients are most vulnerable to infections, so by reducing that time to 11.5 days, we shorten that vulnerable period."

Provided by Duke University Medical Center