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Unrelated Cord Blood Transplant Successful in Children With Shwachman-Diamond Syndrome

2006 JAN 12 - (NewsRx.com) -- Unrelated umbilical cord blood transplantation is successful in children with Shwachman-Diamond syndrome.

"Shwachman-Diamond syndrome (SDS) is an autosomal recessive disorder characterized by pancreatic insufficiency and variable degrees of neutropenia. SDS patients are at risk of developing myelodysplasia, aplastic anemia, and leukemic transformation.

"The role and timing of allogeneic hematopoietic stem cell transplantation (HSCT) in SDS remain controversial," investigators in the United States report.

According to R. Vibhakar and colleagues at the University of Iowa Hospital & Clinic, "We report three SDS patients with severe aplasia transplanted using unrelated umbilical cord blood (UCB).

"Patients received melphalan (180 mg/m²), etoposide (1200 mg/m²), antithymocyte globulin (90 mg/kg), and total lymphoid irradiation (500cGy); graft-versus-host disease (GVHD) prophylaxis consisted of cyclosporine and prednisone."

"Myeloid engraftment occurred promptly with absolute neutrophil count >500cells/mm³ on day 15±5 and all patients displayed 100% donor chimerism by 2 months post-transplant.

"The major complication of transplant was GVHD, with all patients developing grade II or III acute GVHD, one progressing to chronic extensive GVHD. Patients are alive 309, 623, and 2029 days post-transplant," said researchers.

"Factors important in HSCT outcome for SDS may include transplantation at a young age, avoidance of cyclophosphamide, and adequate GVHD prophylaxis. Importantly," Vibhakar concluded, "these cases also suggest that unrelated UCB, in the absence of a matched family member, is an excellent alternative stem cell source for SDS patients undergoing HSCT."

Vibhakar and colleagues published their study in Bone Marrow Transplantation (Successful unrelated umbilical cord blood transplantation in children with Shwachman-Diamond syndrome. Bone Marrow Transplant, 2005;36(10):855-861).

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