

(3602) Cord Blood Transplantation for Lysosomal Storage Diseases Demonstrates the Potential of Cord Blood Cells for Future Cellular Therapies. Session Type: Poster Session, Monday, December 6, 5:30 P.M., Halls DE. Joanne Kurtzberg, William Krivit . Pediatric Blood and Marrow Transplant Program, Duke University Medical Center, Durham, NC, USA; Pediatric Blood and Marrow Transplant Program, University of Minnesota, Minneapolis, MN, USA.

Cord blood stem and progenitor cells can rescue the bone marrow and immune system of pediatric and selected adult patients undergoing myeloablative chemotherapy. Cord blood cells may also be capable of transdifferentiation into non-hematopoietic tissues. Since 1995, >100 infants and children with inborn errors of metabolism have been transplanted with cord blood. The results in these pediatric patients serve as a demonstration that cord blood cells can differentiate into non-hematopoietic tissues.

Thirty five young children with Hurler Syndrome (MPS I) were transplanted with partially HLA mismatched unrelated donor umbilical cord blood over the past 8 years. All had the severe genotype and phenotype. Eighty-seven percent of patients are surviving event-free for a median >3 years. All surviving children remain full donor chimeras and have shown increasing velocities of gains of neurocognitive functions. Skeletal growth improved with only 4/11 children. No child developed clinical cardiac disease and corneal clouding improved in all.

Additional children (n=60) with lysosomal storage diseases including metachromatic leukodystrophy, adrenoleukodystrophy and globoid leukodystrophy (Krabbe Disease), MPS III (Sanfilippo Syndrome) and GM2 (Tay Sachs Disease) have been transplanted with unrelated donor umbilical cord blood over the past 9 years. In asymptomatic children, disease was arrested before the onset of neurologic dysfunction. In symptomatic children disease progression was arrested within 6-9 months of the transplant procedure.

In a child with advanced Krabbe disease who died 1 year post transplant, engraftment of donor cells was noted in the brain. Differentiation to oligodendrocytes was demonstrated in vitro and subsequently in vivo. In a child with MPS III (Sanfillipo syndrome), donor cells differentiated into cardiac myocytes in the heart 6 months post transplant. These studies suggest that cord blood is capable of transdifferentiation into non-hematopoietic lineages. While additional studies are needed to fully define the potential of these cells for cellular therapies and tissue repair, the investigators believe that UCB is a unique stem cell source that will be an important resource for cellular therapies in the future.

From American Society of Hematology Annual Meeting, 2004.

Accessed 11/15/2005.

http://www.cordbloodforum.org/biblio/ash2004/biblio_ash04_child_genetic.html