

**TRANSPLANTATION OF UMBILICAL-CORD BLOOD IN BABIES WITH  
INFANTILE KRABBE'S DISEASE**

Maria L. Escolar, M.D., Michele D. Poe, Ph.D., James M. Provenzale, M.D., Karen C. Richards, M.D., June Allison, R.N., Susan Wood, P.N.P., David A. Wenger, Ph.D., Daniel Pietryga, M.D., Donna Wall, M.D., Martin Champagne, M.D., Richard Morse, M.D., William Krivit, M.D., Ph.D., and Joanne Kurtzberg, M.D.

Editorial  
by Weinberg, K. I.

**ABSTRACT**

**Background** Infantile Krabbe's disease produces progressive neurologic deterioration and death in early childhood. We hypothesized that transplantation of umbilical-cord blood from unrelated donors before the development of symptoms would favorably alter the natural history of the disease among newborns in whom the disease was diagnosed because of a family history. We compared the outcomes among these newborns with the outcomes among infants who underwent transplantation after the development of symptoms and with the outcomes in an untreated cohort of affected children.

**Methods** Eleven asymptomatic newborns (age range, 12 to 44 days) and 14 symptomatic infants (age range, 142 to 352 days) with infantile Krabbe's disease underwent transplantation of umbilical-cord blood from unrelated donors after myeloablative chemotherapy. Engraftment, survival, and neurodevelopmental function were evaluated longitudinally for four months to six years.

**Results** The rates of donor-cell engraftment and survival were 100 percent and 100 percent, respectively, among the asymptomatic newborns (median follow-up, 3.0 years) and 100 percent and 43 percent, respectively, among the symptomatic infants (median follow-up, 3.4 years). Surviving patients showed durable engraftment of donor-derived hematopoietic cells with restoration of normal blood galactocerebrosidase levels. Infants who underwent transplantation before the development of symptoms showed progressive central myelination and continued gains in developmental skills, and most had age-appropriate cognitive function and receptive language skills, but a few had mild-to-moderate delays in expressive language and mild-to-severe delays in gross motor function. Children who underwent transplantation after the onset of symptoms had minimal neurologic improvement.

**Conclusions** Transplantation of umbilical-cord blood from unrelated donors in newborns with infantile Krabbe's disease favorably altered the natural history of

the disease. Transplantation in babies after symptoms had developed did not result in substantive neurologic improvement.

## **SOURCE INFORMATION**

From the Program for Neurodevelopmental Function in Rare Disorders, Clinical Center for the Study of Development and Learning (M.L.E.), and Frank Porter Graham Child Development Institute (M.D.P.), University of North Carolina at Chapel Hill, Chapel Hill; the Pediatric Blood and Marrow Transplant Program, Duke University Medical Center, Durham, N.C. (J.M.P., K.C.R., J.A., S.W., R.M., J.K.); Jefferson Medical College, Philadelphia (D.A.W.); the Pediatric Blood and Bone Marrow Transplantation Program, DeVos Children's Hospital, Grand Rapids, Mich. (D.P.); the Texas Transplant Institute, San Antonio (D.W.); Hôpital Sainte-Justine, Montreal (M.C.); and the University of Minnesota School of Medicine, Minneapolis (W.K.).

Address reprint requests to Dr. Escolar at the Center for the Study of Development and Learning, University of North Carolina at Chapel Hill, Box 7255, Chapel Hill, NC 27599-7255, or at maria.escolar{at}cdl.unc.edu.

This article has been cited by other articles:

Weinberg, K. I. (2005). Early Use of Drastic Therapy. *N Engl J Med* 352: 2124-2126 [Full Text]