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Volume 352:2069-2081

[May 19, 2005](#)

Number 20

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Transplantation of Umbilical-Cord Blood in Babies with Infantile Krabbe's Disease

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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

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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

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