

## TRANSPLANTATION FOR SICKLE CELL DISEASE: Cord Blood as an Alternative to Marrow

The first bone marrow transplantation (BMT) for sickle cell disease was reported in 1984 for a person with both sickle cell anemia and acute leukemia. An NIH supported multi-center study<sup>1</sup> demonstrated that BMT using matched sibling donors can cure sickle cell disease in children. However a major limitation to the use of BMT is the availability of matched sibling donors. The National Marrow Donor Program (NMDP) which was begun in 1987 has access to over one million unrelated volunteers but can identify HLA matched donors for only 30% of all patients. Moreover minorities are underrepresented as donors.

A promising alternative to bone marrow as a source of stem cells for transplantation is umbilical cord blood (UCB). UCB contains sufficient numbers of hematopoietic stem and progenitor cells to reliably effect engraftment. The data also suggest a lower than anticipated risk of acute graft vs. host disease with UCB than with marrow, at least in children.<sup>2,3,4,5</sup> Moreover cord blood is available without a painful invasive procedure.

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### Advantages of Marrow

- ♦ experience (NMPD has facilitated 7,440 transplants through 7/98)
- ♦ higher rate of engraftment
- ♦ donor recall possible
- ♦ lower risk of genetic disease transmission

### Advantages of Cord Blood

- ♦ fewer HLA restrictions
  - ♦ lower risk of graft vs. host disease (GVHD)
  - ♦ lower risk of cytomegalovirus (CMV)
  - ♦ may be banked therefore readily available
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### Free Cord Blood Banking Available

Children's Hospital in Oakland California has developed a **Related Cord Blood Program** for families who have a child with thalassemia, sickle cell anemia, leukemia, aplastic anemia or any other blood disease treatable by BMT. The program offers to test and save cord blood from subsequently born siblings for possible future use for transplantation. The program provides the necessary informed consent and medical history forms, the collection kits, and packing materials, and performs the necessary testing of maternal and cord blood. The cord blood is cryopreserved in liquid nitrogen and stored indefinitely in vapor phase. If a decision to transplant is made it will ship to the transplantation center.

The sender will not be billed for any service provided by the program. However in selected cases Oakland will attempt to recover the cost of these services from third parties. The program invites physicians to refer any family to them whom they think would benefit from the service.

To receive a brochure describing the program or to ask any questions contact:

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#### References:

<sup>1</sup> Walters, M.C., et al., *N Engl J Med* 1996; 335:369-76

<sup>2</sup> Wagner, J.E., et al., *Lancet* 1995; 346:214-19

<sup>3</sup> Kutzberg J., et al., *N Engl J Med* 1996; 335:157-166

<sup>4</sup> Wagner J.E., et al., *Blood* 1996; 88:795-802

<sup>5</sup> Gluckman E., et al., *N Engl J Med* 1997; 337:373-381

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**ROCHESTER**  
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Offers Help

Regarding treatment contact:

Dr. Norma Lerner or Pat Lamarche R.N., P.N.P. Department of Pediatrics 275-2981  
Dr. Karen Kaplan, Department of Medicine 275-3761

Regarding laboratory diagnosis, newborn screening and genetic counseling, contact:

Dr. Peter Rowley, Sandra LaBella or Starlene Loader, Division of Genetics 275-4602

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