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## **CORD BLOOD EDITORIAL BACKGROUND AND FACT SHEET**

Since the late 1980s, umbilical cord blood and related birthing tissues have evolved from medical waste to a rich source of stem cells that can be transplanted to restore normal development and function in blood diseases such as leukemia and lymphoma, inherited genetic disorders, bone marrow failure and immune deficiency diseases.

Preliminary data of clinical studies suggest that progenitor cells contained in umbilical cord blood may be able to repair organ injuries, treat cardiac and vascular disorders, ameliorate immune conditions such as diabetes, and treat neurological disorders including autism, cerebral palsy and spinal cord injury.

### **Stem Cell Donor Alternatives**

The search for a suitable donor for patients who need a hematopoietic stem cell transplant begins within the family. Brothers and sisters with the same parents have a 25% chance of being a match. However, about 70% of patients do not have a match within the family. For them, physicians can search registries around the world for a suitable adult bone marrow donor or banked stem cells derived from donated umbilical cord blood.

Among unrelated hematopoietic stem cell transplants today, cord blood is the source for about 40% in pediatric patients and 10% of adult patients.

### **Advantages/Disadvantages of Cord Blood**

Cord blood as a source of hematopoietic stem cells for transplant has several advantages over adult bone marrow. Cord blood stem cells typically have not been exposed to viruses, chemicals and environment pollutants that can alter cell function. Because of a lower number of immune cells, cord blood stem cells also do not have to be matched as closely to the patient as do cells from adult donors. In addition, cord blood is easily banked so is more quickly available than obtaining stem cells from an adult donor who must be located, consented, tested and harvested. As such, cord blood can be the preferred donor source for patients who have a life-threatening genetic disorder, need a transplant quickly, or have an uncommon tissue type because of their racial or ethnic heritage.

On the other hand, as the number of hematopoietic stem cells in a unit of cord blood is lower than with adult sources, patients undergoing cord blood transplant may take longer to engraft, which can increase the risk of transplant-related mortality and morbidity. These disadvantages are being addressed in current laboratory and clinical research on stem cell expansion and homing.

### **Number of Patients Treated**

More than 35,000 patients have benefited from cord blood transplants since the first transplant in 1989. Currently, cord blood comprises 11% of the stem cell transplant matches facilitated by the National Marrow Donor Program/BeTheMatch registry.

### **Spectrum of Diseases Treated**

At least 80 blood cancers, genetic diseases and immune system and metabolic disorders can be treated or cured with components found in cord blood. (A list of these diseases is available online at <http://www.cb-association.org/indications-for-utilization-of-stored-umbilical-cord-blood>.)

### **First Cord Blood Transplant**

The first cord blood transplant was performed in 1988 at L'Hospital St. Louis in France, using matched sibling cord blood to treat a 5-year-old child with Fanconi anemia. The donor was his newborn sister. The patient is alive and well today, 30 years later.

### **First Unrelated Donor Cord Blood Transplant**

The first cord blood transplant between an unrelated patient and donor was in 1993 – a 4/6 match in a 4-year-old child with T-cell leukemia, performed at Duke University Medical Center.

### **First Cord Blood Transplant in an Adult**

The first cord blood transplant in an adult was in 1995 for a patient with leukemia at Duke University Medical Center. Previously, umbilical cord blood transplants were limited to children because of the relatively small number of stem cells in a unit of cord blood.

### **Public Cord Blood Banks**

The first public cord blood bank was established in 1991 at the New York Blood Center. Around the world today there are more than 700,000 units of cord blood stored in about 160 public banks in 36 countries.

### **Family Cord Blood Banks**

The first family cord blood banks were Cord Blood Registry established in 1992 and Viacord established in 1993. Around the world today more than 5 million units of cord blood are stored in about 210 private or family cord blood banks in 54 countries.

### **Cord Blood Donation Rates**

Cord blood donation rates vary from country to country. In the United States, where nearly 4 million babies are born each year, cord blood is collected for donation in fewer than 5% of births.

### **Cord Blood Registries**

International registries for searching for cord blood unit matches were established by the International NetCord Foundation in 1996 and the National Marrow Donor Program/BeTheMatch in 2001.

### **Regulation of Cord Blood Banks**

Cord blood banking regulations vary widely among countries that have regulations. In Australia, cord blood banks have been regulated under the Australian Therapeutic Goods Administration (TGA) since 2007. In the United States, the Food & Drug Administration (FDA) in 2011 issued a final guidance for licensure of unrelated donor cord blood banks.

### **Cord Blood Banking Standards and Accreditation**

Standards and voluntary accreditation programs for cord blood banks are maintained by AABB and the Foundation for the Accreditation of Cellular Therapy (FACT). Health professionals and expectant parents can look for these bank accreditations.

### **Government Support of Cord Blood Banking**

In the United States, the Stem Cell Act of 2005 created the C. W. Bill Young Cell Transplantation Program that is administered through the Health Resources and Services Administration. It partially funds the National Cord Blood Inventory (NCBI), the National Marrow Donor Program/BeTheMatch cord blood registry, the NMDP's cord blood coordinating center for adult and cord blood donors, NMDP's patient advocacy group and the Stem Cell Transplant Outcomes Database (SCTOD).

### **Multi-Center Clinical Studies**

The first multi-institution, prospective study of unrelated donor cord blood transplantation was the Cord Blood Transplantation Study (COBLT), conducted from 1999-2004.

### **Sickle Cell Anemia**

The first reported success in using related cord blood transplantation to cure children with sickle cell anemia was in 2003.

### **Minimal Cell Dose for Cord Blood Transplants**

A Blood and Marrow Transplant Clinical Trials Network (BMT CTN) 0501 study published in 2009 compared single-cord and double-cord transplants in children with hematological malignancies. It demonstrated that a single cord was adequate when the minimal cell dose was  $2.5 \times 10^7$  total nucleated cells per kilogram of patient weight.

### **Cord Blood Association**

The Cord Blood Association, an international, non-profit association for public and family banking, was established in 2014. Its priorities are quality products and services, advocacy, market expansion, research and development, and public and health professional education.