

CORD BLOOD STEM CELL TRANSPLANTATION

What is stem cell transplantation?

Stem cell transplantation is an accepted form of treatment for patients who require very high-dose chemotherapy or radiation therapy to treat their disease, usually a cancer. This treatment results in severe injury to blood cells formed in the marrow. In order to restore the patient's ability to make blood and immune cells, stem cells from a compatible donor can be administered. Stem cell transplantation is used to treat selected patients with leukemia or lymphoma, as well as some other severe inherited or acquired disorders of the marrow or the immune system.

Bone marrow is used for such transplants because it contains "undifferentiated" cells, called stem cells. Stem cells are very immature cells that can develop into any of the three types of blood cells (red cells, white cells, or platelets). Blood also contains stem cells, but in such small numbers that the cells cannot be counted or identified in ordinary blood tests. Nevertheless, with new procedures, stem cells can be induced to leave the marrow and enter the blood from which they are collected (harvested) for administration to patients who require stem cell transplantation. Stem cells are also present in umbilical cord and placental blood which can be collected after a baby is born.



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Stem cell transplantation (marrow, blood, or cord blood) may be autologous (using the patient's own stem cells, which are collected prior to high-dose chemotherapy) or allogeneic (using stem cells from a related or unrelated, matched donor).

What is cord blood stem cell transplantation?

Blood contained in the placenta and umbilical cord of newborn babies is emerging as a new source of stem cells. Cord blood contains significant numbers of stem cells and has advantages over bone marrow or adult blood stem cell transplantation for certain patients. Umbilical cord blood stem cell transplantation has transformed a waste product of the birth process into a life-saving resource.

What diseases may be treated with cord blood stem cell transplantation?

The first cord blood stem cell transplant was performed in 1988 in Paris, France. The patient was a boy with Fanconi's syndrome (a rare and serious type of anemia) who is alive and healthy today. Since that first transplant, cord blood stem cell transplants have been successfully performed on patients (mostly children) with severe aplastic anemia, Gunther's disease, Hunter syndrome, Hurler syndrome, acute lymphocytic leukemia (ALL), acute myelogenous leukemia (AML), myelodysplasia, chronic myelogenous leukemia (CML), juvenile chronic myelogenous leukemia (JCML),



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neuroblastoma, non-Hodgkin's lymphoma, thalassemia, Wiskott-Aldrich syndrome, and X-linked lymphoproliferative syndrome. To date, more than 800 cord blood stem cell transplants have been performed worldwide. Approximately 75% of these have been done with unrelated donors.

How is cord blood collected, stored, and used for transplantation?

The most commonly used procedure for collecting cord blood is relatively simple. Immediately after a baby is delivered, the umbilical cord is clamped. The baby is then removed from the area and the placenta is placed in a sterile supporting structure with the umbilical cord hanging through the support. The cord is then cleansed with povidone-iodine (Betadine) and alcohol, and a needle is inserted into the umbilical vein. Blood is drawn through the needle into a standard blood collection bag containing nutrients and a solution to keep the blood from clotting (anticoagulant solution). Blood is then collected by gravity drainage, yielding an average 75 milliliters (mL) of blood.

A second method involves collecting the cord blood after delivery of the child, while the placenta is still in the mother. Theoretically, this method may be advantageous by beginning the collection earlier (before the blood has a chance to clot), and by using the contractions of the uterus to enhance blood collection. However, the technique is more intrusive, with the potential to interfere with the mother's care after delivery.



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After collection, the bag of cord blood is immediately transported to a facility for testing and preservation. Testing procedures include tissue typing (a process called HLA-typing) to facilitate matching to potential recipients, as well as testing for infectious agents such as the AIDS virus, cytomegalovirus, and hepatitis viruses. The blood is then frozen and held in liquid nitrogen at very low temperature for future use. One can remove the red cells from the cord blood after collection to reduce the volume of material stored to minimize problems with incompatible red cell administration at the time of administration to the recipient.

At the time of transplant, the cord blood unit is thawed and infused through a vein into the patient.

What facilities are used for the collection of cord blood?

Facilities that collect and store this product are called cord blood banks. It is necessary to distinguish between cord blood banks designed to provide blood from unrelated donors (allogeneic use) from for-profit facilities that offer storage of cord blood in the event it is needed by the donor infant or family member at a later time (autologous use).

Public (not-for-profit) cord blood banks do not charge parents for donating cord blood. Private banks, however, offer cord blood preservation and storage (on a for-profit basis) for the potential use of the donor at a later time, if a condition develops that would benefit from transplantation.



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Does one type of transplant have advantages over another?

Theoretically, allogeneic stem cell transplants may be more successful for patients with certain cancers because of a lower risk of disease relapse than is the case with autologous transplants. However, this is unproven and varies with different disease states.

What are the benefits of cord blood stem cell transplantation?

Potential advantages of cord blood stem cell transplantation over bone marrow transplants include:

- large potential donor pool;
- rapid availability, since the cord blood has been prescreened and tested;
- greater racial diversity can be attained in the banks by focusing collection efforts on hospitals where children of underrepresented ethnic backgrounds are born;
- no risk or discomfort for the donor;
- rare contamination by viruses and
- lower risk of graft-versus-host disease (where the donor's cells attack the patient's organs and tissues), even for recipients with a less-than-perfect tissue match.



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What are the potential disadvantages of cord blood stem cell transplantation?

Cord blood stem cell transplantation is a new, experimental procedure, compared with other sources of stem cells. It is possible that genetic or congenital diseases (those that are present at the time of birth) carried by stem cells, although not immediately apparent, may be transmitted to patients receiving allogeneic cord blood stem cell transplantation. Follow-up procedures to track this possibility would require creating a long-term link between the medical facility caring for the recipient and the donor center and the donor. This procedure has raised concerns about privacy. One solution, which is used by some centers, is to obtain from potential donors complete and detailed questionnaires, prior to cord blood collection. These questionnaires have particular emphasis on individual and family histories of disease, as well as a detailed sexual history. If responses to the questionnaire generate medical concern, the unit is not collected. This technique is similar to that used to screen blood donors.

Another area of uncertainty relates to the requirements for predicting long-term success using stem cells from cord blood. For example, the minimum number of cells required is based on the recipient's weight, age, and disease status. Cord blood might contain too few stem cells for the recipient's size.

Cord blood stem cells take hold more slowly than stem cells from marrow or adult blood. Until



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engraftment occurs, patients are at risk of developing life-threatening infections. So far, however, the incidence of fatal infections in patients receiving cord blood stem cell transplant does not appear to be higher than that seen in other types of stem cell transplantation.

A fourth concern is the potential for contamination of the cord blood with blood from the mother. The mother's blood cells are mature (not stem cells) and they are not of the same HLA type as the cord blood cells. These maternal cells (lymphocytes) could cause severe and even fatal graft-versus-host disease in the transplant recipient.

Finally, the length of time in which cord blood can be stored without losing its effectiveness is not known. Cord blood samples have been preserved and then successfully transplanted for as long as 5 years, thus far. Autologous bone marrow stored longer than 2 years has been successfully transplanted in 94% to 97% of patients. In one of these cases, the marrow had been stored for 11 years. It is not known whether these findings will be true of cord blood as well, but this information is critical to the success of cord blood storage efforts. The supply of cord blood is potentially so great that banks could replenish older samples, circumventing this issue.



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What recommendations can patients follow?

- Healthy parents with healthy children, or couples expecting their first child, can donate their child's cord blood to banks and research programs, if their hospital is actively engaged in public cord blood banking or university-based research programs.
- Parents who have a child or family member with cancer, immune deficiencies, or genetic disease that may benefit from transplantation should discuss with their physicians the potential for cord blood collection and transplantation that is "directed" for use in a specific recipient.
- Selected cord blood banks monitor donor infants up to 2 years after donation. If the cord blood unit has not yet been shipped or used, parents can withdraw their permission to transplant the unit to an unrelated recipient.
- Well before delivery, parents considering private collection should contact their health insurance carrier to find out whether cord blood collection and storage are covered benefits.
- Parents who do not have another child or family member in need of a stem cell transplant must consider the cost associated with private cord blood banking, given the low likelihood that the blood will be used. Companies currently charge approximately \$1000 to \$3000 for collection and storage, with or without an additional yearly storage fee.



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- To minimize the risk to mothers and newborn infants, normal procedures related to delivery should not be altered in order to collect cord blood.
- Because cord blood collection and transplantation are investigational, they should only be done at centers with experience in the transplantation of unrelated sources of stem cells.
- Parents and patients should discuss any questions and concerns with health care professionals who understand cord blood banking and stem cell transplantation.

The following references were used to prepare a portion of this fact sheet.

1. Kline RM, Bertolone SJ. Umbilical cord blood transplantation: providing a donor for everyone needing a bone marrow transplant? *South Med J* 1998; 91(9):821-828.
2. Rubinstein P and others. Outcomes among 562 recipients of placental blood transplants from unrelated donors. *New England Journal of Medicine* 339: 1565, 1998.
3. Smith FO, Kurtzberg J, Karson al. Umbilical cord blood collection, storage, and transplantation: issues and recommendations for parents and patients (unpublished paper).



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Other Resources:

Blood & Transplant Newsletter: phone toll-free at 1-888-433-3313 or 1-847-433-3313.

For cord blood collection sites, contact:

- American Red Cross
- National Marrow Donor Program (NMDP)

More information on cord blood stem cell transplantation:

To get more information, talk to your physician or call The Leukemia & Lymphoma Society, 1-800-955-4572. Web sites that discuss cord blood stem cell transplantation include:

- <http://www.bmtnews.org/newsletters/issue33/cord.html>
- The Leukemia & Lymphoma Society at www.leukemia-lymphoma.org

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