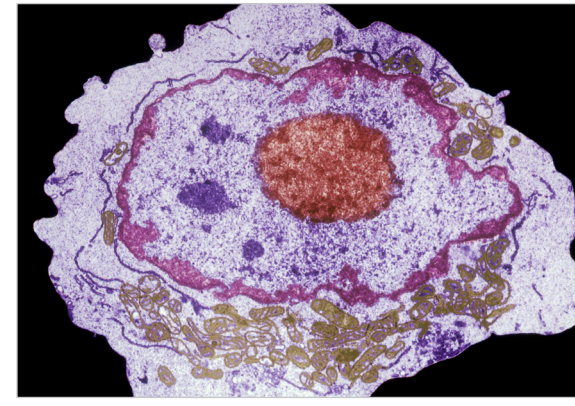
Blood-Forming Stem Cell Transplants



What are bone marrow and hematopoietic stem cells?

Bone marrow is the soft, sponge-like material found inside bones. It contains immature cells known as hematopoietic or blood-forming stem cells. (Hematopoietic stem cells are different from embryonic stem cells. Embryonic stem cells can develop into every type of cell in the body.) Hematopoietic stem cells divide to form more blood-forming stem cells, or they mature into one of three types of blood cells: white blood cells, which fight infection; red blood cells, which carry oxygen; and platelets, which help the blood to clot. Most hematopoietic stem cells are found in the bone marrow, but some cells, called peripheral blood stem cells (PBSCs), are found in the bloodstream. Blood in the umbilical cord also contains hematopoietic stem cells. Cells from any of these sources can be used in transplants.

What are bone marrow transplantation and peripheral blood stem cell transplantation?

Bone marrow transplantation (BMT) and peripheral blood stem cell transplantation (PBSCT) are procedures that restore stem cells that have been destroyed by high doses of chemotherapy and/or radiation therapy. There are three types of transplants:

- In **autologous transplants**, patients receive their own stem cells.
- In **syngeneic transplants**, patients receive stem cells from their identical twin.
- In **allogeneic transplants**, patients receive stem cells from their brother, sister, or parent. A person who is not related to the patient (an unrelated donor) also may be used.

Why are BMT and PBSCT used in cancer treatment?

One reason BMT and PBSCT are used in cancer treatment is to make it possible for patients to receive very high doses of chemotherapy and/or radiation therapy. To understand more about why BMT and PBSCT are used, it is helpful to understand how chemotherapy and radiation therapy work.

Chemotherapy and radiation therapy generally affect cells that divide rapidly. They are used to treat cancer because cancer cells divide more often than most healthy cells. However, because bone marrow cells also divide frequently, high-dose treatments can severely damage or destroy the patient's bone marrow. Without healthy bone marrow, the patient is no longer able to make the blood cells needed to carry oxygen, fight infection, and prevent bleeding. BMT and PBSCT replace stem cells destroyed by treatment. The healthy, transplanted stem cells can restore the bone marrow's ability to produce the blood cells the patient needs.

In some types of leukemia, the graft-versus-tumor (GVT) effect that occurs after allogeneic BMT and PBSCT is crucial to the effectiveness of the treatment. GVT occurs when white blood cells from the donor (the graft) identify the cancer cells that remain in the patient's body after the chemotherapy and/or radiation therapy (the tumor) as foreign and attack them..

What types of cancer are treated with BMT and PBSCT?

BMT and PBSCT are most commonly used in the treatment of leukemia and lymphoma. They are most effective when the leukemia or lymphoma is in remission (the signs and symptoms of cancer have

disappeared). BMT and PBSCT are also used to treat other cancers such as neuroblastoma (cancer that arises in immature nerve cells and affects mostly infants and children) and multiple myeloma. Researchers are evaluating BMT and PBSCT in clinical trials (research studies) for the treatment of various types of cancer.

How is bone marrow obtained for transplantation?

The stem cells used in BMT come from the liquid center of the bone, called the marrow. In general, the procedure for obtaining bone marrow, which is called "harvesting," is similar for all three types of BMTs (autologous, syngeneic, and allogeneic). The donor is given either general anesthesia, which puts the person to sleep during the procedure, or regional anesthesia, which causes loss of feeling below the waist. Needles are inserted through the skin over the pelvic (hip) bone or, in rare cases, the sternum (breastbone), and into the bone marrow to draw the marrow out of the bone. Harvesting the marrow takes about an hour.

The harvested bone marrow is then processed to remove blood and bone fragments. Harvested bone marrow can be combined with a preservative and frozen to keep the stem cells alive until they are needed. This technique is known as cryopreservation. Stem cells can be cryopreserved for many years.

How are umbilical cord stem cells obtained for transplantation?

Stem cells also may be retrieved from umbilical cord blood. For this to occur, the mother must contact a cord blood bank before the baby's birth. The cord blood bank may request that she complete a questionnaire and give a small blood sample.

Cord blood banks may be public or commercial. Public cord blood banks accept donations of cord blood and may provide the donated stem cells to another matched individual in their network. In contrast, commercial cord blood banks will store the cord blood for the family, in case it is needed later for the child or another family member.

After the baby is born and the umbilical cord has been cut, blood is retrieved from the umbilical cord and placenta. This process poses minimal health risk to the mother or the child. If the mother agrees, the umbilical cord blood is processed and frozen for storage by the cord blood bank. Only a small amount of blood can be retrieved from the umbilical cord and placenta, so the collected stem cells are typically used for children or small adults.

How does the patient receive the stem cells during the transplant?

After being treated with high-dose anticancer drugs and/or radiation, the patient receives the stem cells through an intravenous (IV) line just like a blood transfusion. This part of the transplant takes 1 to 5 hours.

What happens after the stem cells have been transplanted to the patient?

After entering the bloodstream, the stem cells travel to the bone marrow, where they begin to produce new white blood cells, red blood cells, and platelets in a process known as "engraftment." Engraftment usually occurs within about 2 to 4 weeks after transplantation. Doctors monitor it by checking blood counts on a frequent basis. Complete recovery of immune function takes much longer, however—up to several months for autologous transplant recipients and 1 to 2 years for patients receiving allogeneic or syngeneic transplants. Doctors evaluate the results of various blood tests to confirm that new blood cells are being produced and that the cancer has not returned. Bone marrow aspiration (the removal of a small sample of bone marrow through a needle for examination under a microscope) can also help doctors determine how well the new marrow is working.

What are the possible side effects of BMT and PBSCT?

The major risk of both treatments is an increased susceptibility to infection and bleeding as a result of the high-dose cancer treatment. Doctors may give the patient antibiotics to prevent or treat infection. They may also give the patient transfusions of platelets to prevent bleeding and red blood cells to treat anemia. Patients who undergo BMT and PBSCT may experience short-term side effects such as nausea, vomiting, fatigue, loss of appetite, mouth sores, hair loss, and skin reactions.

Potential long-term risks include complications of the pretransplant chemotherapy and radiation therapy, such as infertility (the inability to produce children); cataracts (clouding of the lens of the eye, which causes loss of vision); secondary (new) cancers; and damage to the liver, kidneys, lungs, and/or heart.

The likelihood and severity of complications are specific to the patient's treatment and should be discussed with the patient's doctor.

Please refer to the online version of this fact sheet for Selected References and additional material.

Source: National Cancer Institute. ■