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## **What is Stem Cell/Bone Marrow Transplantation?** [1]

This section has been reviewed and approved by the [Cancer.Net Editorial Board](#) [2], 11/2013

 Watch the [Cancer.Net Video: Bone Marrow and Stem Cell Transplantation: An Introduction, with Sonali Smith, MD](#) [3], adapted from this content.

### **Key Messages:**

- A stem cell transplant (sometimes called a bone marrow transplant) is a medical procedure in which diseased bone marrow is replaced by highly specialized stem cells that develop into healthy bone marrow.
- There are two main types of stem cell transplants: autologous, in which the patient receives his or her own stem cells, and allogeneic, in which stem cells are donated by another person.
- Transplantation is a process that has several phases; your specific treatment plan can take weeks or months to complete.
- Talk with your doctor or another member of your health care team about stem cell transplantation so you can make informed decisions about your treatment plan.

Stem cell transplantation is a procedure that is most often recommended as a treatment option for people with leukemia, multiple myeloma, and some types of lymphoma. It may also be used to treat some genetic diseases that involve the blood.

During a stem cell transplant diseased bone marrow (the spongy, fatty tissue found inside larger bones) is destroyed with chemotherapy and/or radiation therapy and then replaced with highly specialized stem cells that develop into healthy bone marrow. Although this procedure used to be referred to as a bone marrow transplant, today it is more commonly called a stem cell transplant because it is stem cells in the blood that are typically being transplanted, not the actual bone marrow tissue.

### **The purpose of bone marrow and hematopoietic (blood-forming) stem cells**

Bone marrow produces more than 20 billion new blood cells every day throughout a person's life. The driving force behind this process is the hematopoietic (pronounced he-mah-tuh-poy-ET-ick)

stem cell. Hematopoietic stem cells are immature cells found in both the bloodstream and bone marrow. These specialized cells have the ability to create more blood-forming cells or to mature into one of the three different cell types that make up our blood. These include red blood cells (cells that carry oxygen to all parts of the body), white blood cells (cells that help the body fight infections and diseases), and platelets (cells that help blood clot and control bleeding). Signals passing from the body to the bone marrow tell the stem cells which cell types are needed the most.

For people with bone marrow diseases and certain types of cancer, the essential functions of red blood cells, white blood cells, and platelets are disrupted because the hematopoietic stem cells don't mature properly. To help restore the bone marrow's ability to produce healthy blood cells, doctors may recommend stem cell transplantation.

## Types of stem cell transplantation

There are two main types of stem cell transplantation:

**Autologous transplantation (AUTO).** A patient undergoing an AUTO transplant receives his or her own stem cells. During the AUTO transplant process, the patient's stem cells are collected and then stored in a special freezer that can preserve them for decades. Usually the patient is treated the following week with powerful doses of [chemotherapy](#) [4] and/or [radiation therapy](#) [5], after which the frozen stem cells are thawed and infused into the patient's vein. The stem cells typically remain in the bloodstream for about 24 hours until they find their way to the marrow space, where they grow and multiply, beginning the healing process.

**Allogeneic transplantation (ALLO).** A patient undergoing an ALLO transplant receives stem cells donated by another person. As a result, the first step for an ALLO transplant is to find a donor match. Specific proteins, called human leukocyte antigens (HLA), are found on the surface of white blood cells and throughout the body. The combination of these proteins makes each person's tissue unique. HLA typing is a special blood test that identifies these proteins. A successful bone marrow transplant requires the donation of near-perfect HLA-matched bone marrow. HLA-matched blood stem cells given to a person during transplantation are less likely to result in [graft-versus-host disease](#) [6] (GVHD, a complication in which the immune cells in the transplanted bone marrow recognize the recipient's body as foreign and attack it). Siblings (brothers or sisters) usually have the best chance of being a complete match. Occasionally, other family members can be a match. In some cases, an unrelated volunteer donor may be the best match. Learn more about [donating bone marrow](#) [7].

Once a donor has been identified, stem cell donation is coordinated so it occurs as close to the end of the patient's initial chemotherapy and/or radiation therapy as possible. On the day of the transplant, the patient receives the unfrozen donated stem cells through an IV that delivers them into his or her vein.

## Comparing AUTO and ALLO transplants

ALLO transplants have two advantages over AUTO transplants. First, with an ALLO transplant, the patient receives cancer-free stem cells because they come from a healthy donor. With an AUTO transplant, there is always the risk that the frozen stem cell product will contain some

remaining cancer cells.

In addition, with AUTO transplants, the cancer-killing effect ends when the chemotherapy and/or radiation therapy ends. The remainder of the treatment helps patients recover from side effects. In contrast, the ALLO transplant recreates the immune system, which continues to attack any remaining cancer cells after the completion of chemotherapy and/or radiation therapy. This effect (called the graft-versus-tumor effect) is especially strong with some cancers.

However, a patient receiving an ALLO transplant faces tissue rejection risks that a person who received an AUTO transplant does not.

### **Choosing between AUTO and ALLO transplants**

The choice of transplant depends on many factors, including the type of disease being treated, the health of the patient's bone marrow, the extent of bone marrow damage caused by previous chemotherapy, and the age and overall health of the patient. In general, people with cancerous or badly diseased marrow tend to receive an ALLO transplant because AUTO transplantation is not recommended.

Making this decision is complex and requires the input of a doctor who specializes in transplant medicine. This means the patient, as well as a potential donor, may need to travel to a center [8] that performs many transplantation procedures to talk with a specialist and have an in-person evaluation. A patient should also consider other nonmedical factors, such as his or her support system, the ability to take time away from work or family, insurance coverage, and access to transportation.

### **Investigational transplants**

Other types of transplants have been developed to meet the needs of patients who cannot receive ALLO or AUTO transplants, and they are currently being researched in clinical trials. These types of transplants may help patients who cannot find a perfectly matched sibling or unrelated donor or patients who cannot be given the intense chemotherapy and/or radiation therapy that is required before ALLO and AUTO transplants. These new approaches to stem cell transplantation include:

**Umbilical cord blood transplantation.** For people who cannot find a matched donor, umbilical cord blood transplantation may be an option. Although still a relatively new source of stem cells, cord blood units are used at cancer centers around the world. The results in children have been promising. In adults, the technology is still underdeveloped, and patients may face additional risks that should be discussed with the doctor. Learn more about donating umbilical cord blood [9]

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**Mini-transplantation (nonablative or reduced-intensity conditioning transplantation).** For a mini-transplant, the treatment before the transplantation of donor cells is less intense, usually resulting in fewer side effects. For example, there may be no hair loss, fewer blood transfusions, and less time spent in the hospital. Large transplant centers offer this approach for patients who meet specific criteria, such as older patients with slow-growing cancers. However, it may not be as effective for patients with a fast-growing cancer.

**Parent/child and haplotype mismatched stem cell transplantation.** This rare type of transplantation is not performed by most centers in the United States because of the high complication rate. However, when no matched donors can be easily found, it may be the only option. For this type of transplant, the donor is a family member who is only a 50% match, rather than the desired nearly 100% match. Parents, children, or siblings may be donors. Because of the incomplete match, complications such as GVHD, infections, and failure of donor cells to grow, happen much more often than with matched-donor transplantation.

## **Transplantation timeline**

The timing of bone marrow transplantation is often misunderstood. Many people are surprised to learn that it is a process that takes weeks or even months from beginning to end. The following timelines lay out the major steps for AUTO and ALLO transplants. One phase usually leads directly into the next, but some phases (such as stem cell collection) may be done in advance. Talk with your doctor to learn about the specific timeline for your treatment.

### ***AUTO transplant timeline***

#### Phase I: Patient blood stem cell collection

- This involves surgical placement of a transplant catheter (a thin tube temporarily put into a large vein to make injections easier), injections of white blood cell growth factor (medication to increase white blood cell levels, which helps the body fight infections), and removing bone marrow or taking blood through apheresis (the process of giving only select blood components: platelets, plasma, red cells, granulocytes, or a combination of these).
- Overall time: One to two weeks
- Treatment setting: Outpatient (does not require hospital admission; can take place at a hospital building, a clinic, or a doctor's office)

#### Phase II: Transplant treatment

- This involves high-dose chemotherapy and/or radiation therapy.
- Overall time: Five to seven days
- Treatment setting: Outpatient or inpatient (requires admission to a hospital)

#### Phase III: Stem cell transfusion/infusion

- This involves transfusion of stem cells through an IV, which takes about one hour.
- Overall time: One day
- Treatment setting: Outpatient or inpatient

#### Phase IV: Recovery

- This involves antibiotics, growth factors, blood transfusions, and managing any side effects from the procedure.
- Overall time: Two weeks
- Treatment setting: Outpatient or inpatient

#### ***ALLO transplant timeline***

##### Phase I: Donor blood stem cell collection

- This involves injections of white blood cell growth factor and the removal of bone marrow or blood collection through apheresis.
- Overall time: One week
- Treatment setting: Outpatient

##### Phase II: Transplant treatment

- This involves high-dose chemotherapy and/or radiation therapy.
- Overall time: Five to seven days
- Treatment setting: Outpatient or inpatient

##### Phase III: Stem cell transfusion/infusion

- This involves transfusion of stem cells through an IV, which takes about one hour.
- Overall time: One day
- Treatment setting: Outpatient or inpatient

##### Phase IV: Recovery

- This involves medications, including those that suppress the immune system, antibiotics, and growth factors, and blood transfusions and fluids to help manage the patient's symptoms.
- Overall time: Eight to 12 weeks
- Treatment setting: Mostly outpatient, some inpatient

#### **Milestones of successful transplantation**

"Success" means different things to different people. Two common milestones patients, their families, and doctors use to measure success are:

**Recovery of blood counts to safe levels.** Most types of transplantation cause blood counts to fall from normal levels to near zero for a period of one to two weeks. During this time, there is danger of infection from low numbers of white blood cells, danger of bleeding from low numbers of platelets, and danger of fatigue from low numbers of red blood cells. These risks are reduced by using transfusions and antibiotics during this time. The true measure of recovery is the return of blood counts to normal levels through the multiplying of stem cells.

**Cure of the disease.** The intent of stem cell transplantation is to cure the disease. For some

types of cancer, prolonged remission (time spent without any signs of cancer) is the best result, but for many serious cancers of the marrow, such as acute leukemia and lymphoma, a permanent cure is possible. However, there is no way to know for sure the cancer is permanently gone, so patients are encouraged to have regular follow-up care and testing to watch for signs that the cancer has come back.

## **Questions to ask the doctor**

Talking often with the doctor [10] is important so you can make informed decisions about your health care. The following questions may help you learn more about stem cell / bone marrow transplantation. You are also encouraged to ask additional questions that are important to you.

- Which type of stem cell transplant would you recommend? Why?
- If I will be having an ALLO transplant, how will we find a donor? What is the chance of finding a good match?
- Will I receive chemotherapy and/or radiation therapy before the transplant?
- Can you explain the expected timeline for my treatment?
- How long will I need to stay in the hospital?
- How will this treatment affect my daily life? Will I be able to work, exercise, and perform my usual activities?
- How will we know if the transplant is working?
- What will happen if the transplant doesn't work or the cancer comes back?
- What short-term and long-term side effects may be associated with this treatment?
- What follow-up tests will I need and how often will I need them?
- If I'm worried about managing the costs related to my cancer care, who can help me with these concerns?

## **More Information**

Bone Marrow Aspiration and Biopsy [11]

Making Decisions About Cancer Treatment [12]

Managing the Cost of Cancer Care [13]

ASCO Cancer Treatment Plans and Summaries [14]

## **Additional Resources**

Explore BMT [15]

Be the Match: National Marrow Donor Program [16]

Blood & Marrow Transplant Information Network [17]

U.S. Department of Health and Human Services: Understanding Transplantation as a Treatment Option [18]

## National Bone Marrow Transplant Link [19].

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### **Links:**

- [1] <http://www.cancer.net/navigating-cancer-care/how-cancer-treated/bone-marrowstem-cell-transplantation/what-stem-cellbone-marrow-transplantation>
- [2] <http://www.cancer.net/about-us>
- [3] <http://www.cancer.net/node/27351>
- [4] <http://www.cancer.net/node/24473>
- [5] <http://www.cancer.net/node/24661>
- [6] <http://www.cancer.net/node/24674>
- [7] <http://www.cancer.net/node/24502>
- [8] <http://www.cancer.net/node/24706>
- [9] <http://www.cancer.net/node/24503>
- [10] <http://www.cancer.net/node/24958>
- [11] <http://www.cancer.net/node/24409>
- [12] <http://www.cancer.net/node/24582>
- [13] <http://www.cancer.net/node/24865>
- [14] <http://www.cancer.net/node/25394>
- [15] <http://www.explorebmt.org/>
- [16] <http://bethematch.org/>
- [17] <http://www.bmtinfonet.org/>
- [18] [http://bloodcell.transplant.hrsa.gov/transplant/understanding\\_tx/index.html](http://bloodcell.transplant.hrsa.gov/transplant/understanding_tx/index.html)
- [19] <http://www.nbmtlink.org/>