

Part 2: The Clinical Applications of Donating and Banking Stem Cells



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# Storing Stem Cells for Future Use

#### **AN OVERVIEW**

Stem cells function in some of our tissues throughout life to replenish normal cellular wear and tear and respond to stress and disease. These specialized cells can replicate as well as give rise to more specialized cells. Some types of stem cells can also be stored for future clinical application. These cells can be frozen and banked for personal use or donated to a patient in need.

Just like you can donate blood or organs, you can donate stem cells, a powerful act that has the potential to save someone's life. For example, donated blood stem cells can be transplanted into a patient with blood or immune system diseases, such as some blood cancers or immunodeficiencies, and provide a potential cure.

Some types of stem cells can also be frozen at very low temperatures and stored, or banked, for future use. For example, blood stem cells can be banked and used for someone who needs a future transplantation. In this resource, we provide the current science on storing several commonly discussed stem cell types.

#### **DONATING BLOOD STEM CELLS**

## Why Donate

Blood stem cells make all of the blood and immune cells for a person for their entire life (see Figure 1). Unfortunately, in some diseases, blood stem cells fail and they need to be replaced in order for the patient to survive. Through blood stem cell transplantation, healthy blood stem cells can replace diseased ones, potentially making all future blood and immune cells and curing the patient. Blood stem cells can be obtained from the placenta and umbilical cord of newborns, and from the peripheral blood or bone marrow of adults.

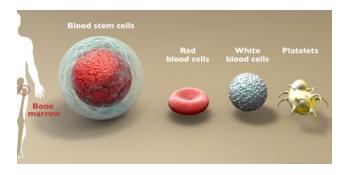


Figure 1: Blood stem cells, which reside in the bone marrow, make all types of cells of the blood and immune system, including red blood cells, white blood cells, and platelets.

Blood stem cell transplantations are most commonly used to treat blood diseases (such as cancers or red blood cell disorders), bone marrow failure diseases, and certain diseases that result from missing or dysfunctional immune cells. Inherited metabolic diseases (deficiencies in breaking down substances in the body) can also be treated by transplantation. More about the specific diseases that can be treated by blood stem cell transplantation can be found at Be the Match.

# How to donate

A donor and patient must be an immune match to avoid transplant rejection (see <u>Matching Donors and Patients for Transplantation</u>). The ideal donor is a matched relative, but this can only be found for about 25% of patients. The other 75% of patients may find a matched volunteer donor in a stem cell registry.

Stem cell registries contain the genetic information of millions of potential volunteers who can be connected with a patient (see Figure 2). Donors can provide a genetic sample by simply rubbing a sterile swab along the inside of their cheek for 10 seconds. DNA is then isolated from these cheek cells, which is analyzed to identify the person's immunological markers, revealing who they are immunologically compatible with. Matched stem cells can be collected from the volunteer and transplanted to the patient.









Figure 2: A. A potential donor gives a genetic sample by a cheek swab. Her DNA is then isolated, immunological markers are determined, and the information is entered into a stem cell registry. B. After it is determined that she is an immunological match with a patient looking for blood stem cells, the registry member is contacted to let her know she is eligible to donate. C. The donor's blood stem cells are collected to transfer to patient. D. The patient undergoes a blood stem cell transplant to receive the donor's blood stem cells in hopes of disease treatment.

It is important for people of diverse genetic backgrounds to join these registries so that patients have the highest chance of finding stem cells that are a good match. There are global organizations, such as the World Marrow Donor Association and Be the Match, that coordinate global databases of blood stem cell donors to help patients find stem cells for transplantation.

To join a bone marrow registry, contact an organization such as <u>Be the Match</u>, the largest international registry of blood stem cells. More information about donating blood stem cells or becoming part of a registry can also be found through the <u>European Society for Blood and Marrow Transplantation</u>, the <u>Center for International Blood</u> & <u>Marrow Transplant Research</u>, the <u>Asian Pacific Blood and Marrow Transplantation Group</u>, and the <u>Australian Bone Marrow Donor Registry</u>.

#### **Blood Stem Cell Collection**

Blood stem cells for transplantation can be collected in several different ways. After a baby is delivered, blood stem cells can be collected from cord blood—the blood found in the placenta and umbilical cord (to learn more see <a href="Cord Blood">Cord Blood</a> & Uses to Treat Disease).

Blood stem cells also can be collected later in life. In adults these stem cells reside in the soft, spongy area of bone, known as bone marrow. Blood stem cells can be collected directly from the bone marrow, but more often are mobilized into the blood stream

and collected from a vein in the arm. Stem cells from either source can be collected for personal or family use or donated to a patient.

Of note, blood stem cell transplantation has only been shown to treat certain blood, immune, and metabolic diseases. Clinical trials are ongoing to evaluate blood stem cell transplantation for other types of disease, but consumers should be wary of providers advertising the use of blood stem cell transplantation to treat other diseases.

# MATCHING DONORS AND PATIENTS FOR TRANSPLANTATION

Your immune system is constantly taking stock of the cells in your body to determine whether cells belong to you and should be left alone, or if they are foreign and should be attacked. It is critical that if you receive a transplantation that you do not mount an immune attack, which could kill the new cells or tissue, leading to transplant rejection. There is an additional complication when the cells that are donated produce their own immune system. In this case the transplanted immune cells could mount an attack on the patient's own tissues, which can be life-threatening. This is known as graft-versushost-disease. In order for a patient to receive a blood stem cell transplant the stem cells must be immunologically compatible, or matched, so that they will not attack the patient's tissues.

#### **CELL BANKING**

In addition to donating stem cells for immediate use, stem cells can be banked for use in the future. Cells, such as cord blood stem cells, can be frozen at very low temperatures and stored in public banks for donation or in private banks, for personal or family use.

Organizations such as <u>Be the Match</u> have public cord banks, which can match this life-saving resource



with patients in need. A list of public cord blood banks around the world can be found at Parent's Guide to Cord Blood Foundation. At this time the Council of Europe European Committee on Organ Transplantation, American Academy of Pediatrics, the American College of Obstetricians and Gynecologists, and the World Marrow Donor Association recommend that families donate cord blood to public cord blood banks, rather than storing it in a private bank for "biological insurance" against a future issue.

If you choose to store cord blood in a private bank you should ensure that the bank has been accredited by a reputable organization, such as the Foundation for the Accreditation of Cellular Therapies (FACT) or AABB, two organizations that set international standards. To learn more about banking cord blood for public or private use see Cord Blood and Uses to Treat Disease: Private Banking vs Public Donation.

### SHOULD YOU BANK STEM CELLS FOR PERSONAL USE?

Patients should be aware that there are unscrupulous stem cell clinics that make false promises about stem cell therapies and promote banking stem cells for years, or even decades, for unproven future uses, if they can even be used at all. Additionally, any offered "treatments" could cause physical and financial harm. Typically, there are yearly storage fees at private banks, and this storage of cells for a potential treatment that has not yet been proven safe or effective can be costly. At this time, it is not recommended to bank these stem cells:

### **Blood Stem Cells for Unproven Purposes**

There are companies that promote banking adult blood stem cells for new, experimental personal uses. These companies claim that stem cells can be frozen when you are young and used later in life for "rejuvenation," or for "treatments" claiming to restore youthfulness and vitality. Currently, there is no evidence, however, that such a "treatment" would be effective in humans. Recently, the U.S. Food and Drug Administration issued a <u>statement</u> warning that infusing young blood into an older individual has no proven clinical benefit,

and that it might in fact cause harm. More research is needed to determine whether transplanting young blood stem cells or blood products into older people can be safe and efficacious.

Consumers should be aware that banking adult blood stem cells for an indeterminate time can be expensive and that unproven stem cell treatments carry medical risk.

# Mesenchymal Stromal Cells (MSCs)

A growing number of clinics and online companies advertise the use of <u>mesenchymal stromal or stem</u> <u>cells</u> (MSCs) for myriad unproven stem cell treatments. Companies try to convince consumers that they should collect and freeze these cells to have on hand to treat a long list of potential future diseases.

While legitimate mesenchymal stem cells were first discovered in bone marrow through rigorous scientific study, the cells that are typically marketed by clinics that promote unproven or unapproved "treatments" are a collection of mixed and undefined cells from a patient's blood, fat, or bone marrow. These cells are marketed for "stem cell" or "MSC" therapies, but the biological properties and therapeutic potential of these uncharacterized cells is unknown, and claims of therapeutic benefit are not supported by sound science. Directly injecting these ill-defined cells into patients is currently controversial and requires further study.

Mesenchymal stem cells also have been reported to be isolated from other tissues, such as dental pulp, amniotic fluid, and cord blood. Some of these cells are thought to have stem cell properties and currently are being studied and may, at some point in the future, have application for disorders. However, there is no known benefit that currently has been identified.

There are multiple legitimate clinical trials underway testing the benefits of rigorously characterized MSCs to treat various diseases and conditions, but it is too early to know if there will be a scientifically proven therapeutic benefit.

Before banking any tissues or cells for a potential future MSC therapy, you should talk to a board certified and licensed physician who is unaffiliated with the stem cell bank or clinic, and is knowledgeable about the patient's condition and the science behind the clinical application of stem cells. Banking these cells, and the treatments offered, can be expensive and there is little to no evidence to date to indicate that they are beneficial. In fact, in some cases they may cause harm.

#### T Cells

T cells are a type of immune cell that are being developed into a cancer therapy that shows great promise in treating various types of the disease. T cells can be collected from a patient and engineered to specifically attack cancer cells, while leaving other cells in the body unharmed. This technology is called chimeric antigen receptor T-cell (CAR-T) therapy. Several CAR-T therapies are approved to treat certain kinds of blood cancers, such as leukemia and lymphoma, in multiple countries and regions around the world.

Some companies are freezing patient T cells for potential future therapeutic use. These companies are making unfounded promises and are offering commercial services that are not fully developed. Additionally, the chances of acquiring one of the types of cancer that can be treated is low.

The <u>International Society for Cell and Gene Therapy</u> recently issued a statement warning patients against banking T cells for potential future use.

#### **BEFORE YOU BANK**

When evaluating any potential therapeutic intervention, you should ask questions and ensure to the extent possible that the treatment has been evaluated in an approved clinical trial, and that claims were tested through rigorous evidence-based science. Whether or not to donate or store stem cells for medical purposes is a personal decision, and you should always consult your doctor or a trusted medical professional.

If you are considering saving stem cells for a potential future stem cell therapy, refer to Nine Things to Know About Stem Cell Therapies to help inform your decision. If you are concerned that a stem cell clinic is providing unproven or unapproved stem cell treatments, you can refer to the How to Report False Marketing Claims and Adverse Events from Clinics Offering Unapproved Stem Cell "Therapies" webpage.

#### **IN SUMMARY**

Recommendations regarding stem cell banking and donation vary by cell type and treatment. Donated blood stem cells are an extremely valuable resource for patients who need blood stem cell transplantations to treat a variety of blood and immune system diseases. Physicians and patients should be aware, however, that there are unscrupulous private banks that make unsubstantiated claims about potential future uses of banked stem cells for other purposes. Before banking any cells for future use, patients should consult a physician and make sure that claims are supported by scientific data. For more information, see <a href="Stem Cell Treatments: What to Ask">Stem Cell Treatments: What to Ask</a>. To learn more about the potential use of stem cells visit A Closer Look at Stem Cells.

#### **USEFUL LINKS**

Asian Pacific Blood and Marrow Transplantation Group

Be the Match

Center for International Blood & Marrow
Transplant Research

European Society for Blood and Marrow Transplantation

**World Marrow Donor Association**