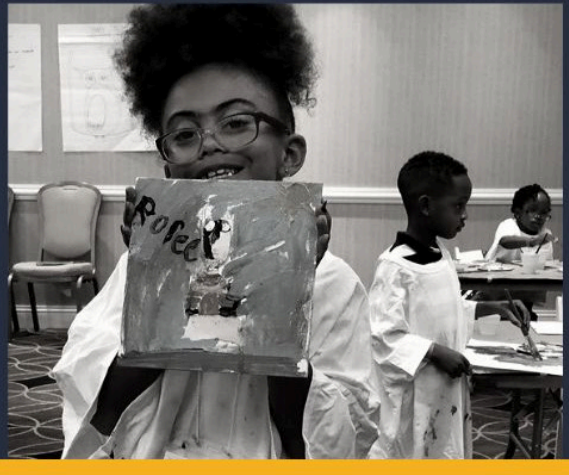
Blood and Marrow Transplant (BMT) for Sickle Cell Disease



Blood and marrow transplant (BMT) is a proven cure for sickle cell disease.

THIS HANDBOOK WAS DEVELOPED TO ANSWER COMMON QUESTIONS ABOUT BMT. PLEASE SHARE ANY QUESTIONS YOU MAY HAVE AFTER REVIEWING THIS BROCHURE WITH YOUR HEALTH CARE TEAM.

CONTENTS

HISTORY OF BMT FOR SICKLE CELL

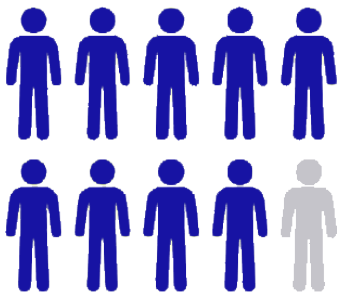
- 1980s: First patient cured of sickle cell disease through BMT. This child also had leukemia and BMT cured both her leukemia and sickle cell disease.

- 1990s: First international clinical trial of BMT for sickle cell disease.

- BMT for sickle cell disease has been done for over 30 years. During this time we have learned how to better support patients during transplant.

- A recent study reported the outcomes of 1000 patients who had BMT with a matched sibling donor for sickle cell disease.

- Younger patients (less than 16 years of age) and patients transplanted more recently (after 2007) had better outcomes.



On average, every 10 children who underwent matched sibling BMT, 9 were cured.

IS BMT FOR ME OR MY CHILD

In the past, only children who had major complications from their sickle cell disease (such as a stroke) were eligible for BMT. Since we know most children do very well after BMT using a matched sibling donor, more children are undergoing BMT today.

In addition, we are now learning that adult sickle cell patients can tolerate BMT better than we expected with positive results, particularly those with a matched sibling donor.

Most children and many adults with sickle cell disease may be eligible for BMT if they have a matched sibling donor.

What if I or my child do not have a matched sibling?

BMT using other donors (parents, unrelated donors) is currently only done in research studies. Research studies have rules on who can participate in the study.

The decision to undergo a BMT can be very difficult. It is important to talk with your health care team and family about treatment options.

BENEFITS OF BMT

CURE

Improved Quality of Life

After BMT a patient may have more energy and feel better.

Organ damage from SCD is decreased or eliminated. For example, in most children the spleen is more efficient in filtering the blood after a BMT.

While additonal damage is prevented; permanent damage to organs are irreversible.

RISKS OF BMT

- **SHORT-TERM SIDE EFFECTS**

Chemotherapy given in preparation for the BMT can cause nausea, vomiting, diarrhea, mouth sores, and hair loss. Blood cell counts also become low. If less chemotherapy is given, then fewer side effects may occur.

- **INFECTION**

After BMT, the immune system is weak and therefore susceptible to infection. A patient needs to take medicines and follow special instructions to lower the chances of an infection.

- **REJECTION**

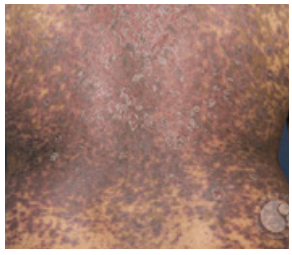
The immune may reject the new donor cells. If this happens, sickle cell disease returns.

- **LONG-TERM SIDE EFFECTS**

Chemotherapy given during preparation for BMT can cause infertility (Inability to have children), and may cause effects on other organs (heart, lungs, liver, kidneys, etc). If less chemotherapy is given, then these side effects may be less likely to occur. We are still learning about the long-term side effects of BMT for sickle cell disease.

- **GRAFT VS HOST DISEASE**

GVHD is rare. Following a BMT, GVHD is a complication that could occur in which the patient's body is attacked by the donor bone marrow. GVHD can be mild but sometimes it can be severe. It can involve the skin, the gut, the liver, and other parts of the body. It can usually be treated, but sometimes it does not go away with treatment.



- **DEATH**

Unfortunately some patients have succumb to complications of BMT.

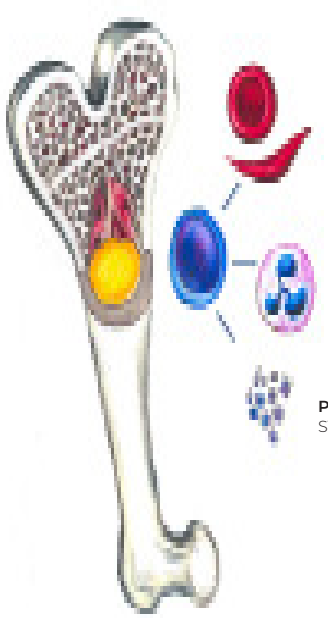
BMT BASICS

BMT IS NOT A SURGERY.

BLOOD AND MARROW TRANSPLANTATION (BMT) IS NOT LIKE OTHER TRANSPLANTS. BMT IS A PROCESS TO REPLACE A PATIENT'S BONE MARROW WITH A DONOR'S BONE MARROW.

BONE MARROW IS THE FACTORY THAT MAKES BLOOD CELLS.

BONE MARROW IS LOCATED WITHIN THE BONES. THE BONE MARROW CONTAIN BLOOD STEM CELLS WHICH ARE SPECIAL CELLS THAT GROW TO BECOME RED BLOOD CELLS, WHITE BLOOD CELLS OR PLATELETS.



RED BLOOD CELLS
CARRY OXYGEN TO THE ENTIRE BODY. IN PEOPLE WITH SICKLE CELL DISEASE, THE RED BLOOD CELLS CAN CHANGE INTO THE SHAPE OF A BANANA. THESE "SICKLE" CELLS DO NOT WORK NORMALLY.

WHITE BLOOD CELLS
FIGHT INFECTION.

PLATELETS
STOP BLEEDING.

FINDING A DONOR: HLA TYPING

Locating a donor is the first step when considering BMT. In general, the best donor is a full sibling. A full sibling is a brother or sister who shares the same mother and father.

What is HLA?

HLA stands for Human Leukocyte Antigen. An HLA type is a blood type for your immune system. A BMT works best if a donor is an HLA-match.

How is HLA typing done?

A blood sample or mouth swab is sent for testing. We recommend testing all healthy full siblings and parents. HLA typing results are usually available within a few weeks.

Will a sibling be an HLA-match?

Each full sibling has a 25%, or one in four, chance of being an HLA-match. This means that some siblings may be a match, but many siblings will not be a match. If a sibling is a blood type match, he or she may or may not be an HLA-match.

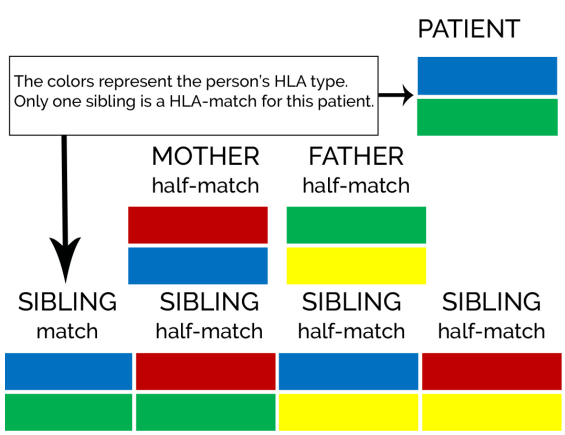
Parents usually are not a match for their children.

What if there is no matched sibling?

It is possible to use an "alternative donor." Alternative donors include:

- unrelated (non-family) HLA-matched volunteer
- donated umbilical cord blood
- half-matched (haploidentical) family member

Transplant using alternative donors for sickle cell disease is currently only done in research studies.



TRANSPLANT

WORK-UP

1 TO 3 MONTHS BEFORE BMT

A patient has tests to check how different organs are working to make sure the patient's organs are healthy enough for BMT. The patient also has a minor surgery to get a central line.

PREP

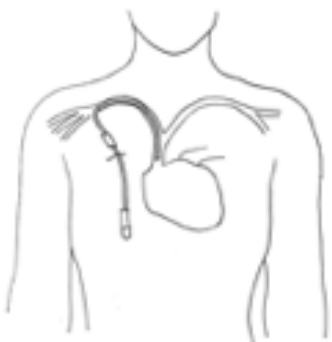
1 TO 3 WEEKS BEFORE BMT

During preparation, a patient is admitted to the hospital and receives "conditioning" medicines. These medicines make room in the bone marrow and turn down the immune system to prevent rejection of the new donor cells. Conditioning usually involves chemotherapy, but we may be able to decrease or not use chemotherapy for some transplants.

TRANSPLANT

DAY 0

Day 0 is transplant day. On this day a patient receives the donor's stem cells through their central line. This is like a blood transfusion.



CENTRAL LINE

A central line is inserted into a blood vessel that travels to the heart. Medicines and blood transfusions can be given through it. Labs can be drawn from it.

TIMELINE

ENGRAFTMENT

2 TO 6 WEEKS AFTER BMT

After transplant, a patient waits for the stem cells from the donor to start making new blood cells. During this time the immune system is very weak and the patient can get an infection. A patient also needs to receive red blood cell and platelet transfusions. When the bone marrow starts making new blood cells, a patient is "engrafted." A patient usually remains in the hospital for a month after the transplant if there are no major problems.

EARLY AFTER BMT

1 TO 6 MONTHS AFTER BMT

During this time a patient is usually out of the hospital but needs to come to the BMT clinic often for visits. Initially there will be many visits and later less visits are needed. A patient must continue to take medicines to make sure the transplant works. The immune system is still weak so a patient should not go to school and should avoid large crowds.

LATE AFTER BMT

6 MONTHS TO YEARS AFTER BMT

Most patients are able to decrease or stop some medicines by 6 months after BMT, and most patients are able to stop all medicines by 12 months after BMT. A patient has a brand new immune system after BMT; therefore, all childhood vaccines must be repeated. Even years after BMT, a patient continues to be followed in either regular BMT clinic or a BMT late effects clinic to monitor for long-term effects.

BMT is a long process that involves six stages

THE DONOR

BONE MARROW

- Bone marrow inside the hip bone is collected using a needle. This is done in the operating room with anesthesia (medicine to make the donor sleep and not feel pain during the procedure). Most donors who donate bone marrow can return to daily activities within 1-2 days after donation.

PERIPHERAL BLOOD

- A donor is administered an injection, usually daily for five days prior which contains G-CSF. This medicine makes the stem cells leave the bone marrow and go out into the blood. This shot is usually given daily for 5 days. The stem cells are then collected with a special machine through a type of IV line.

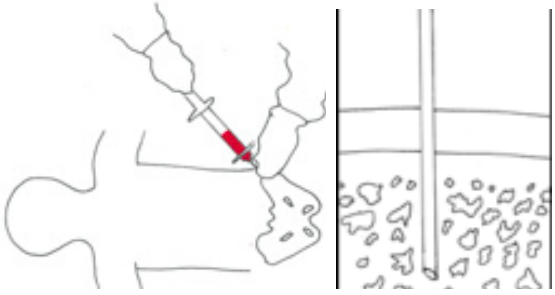
UMBILICAL CORD

- After a baby is born, blood can be collected from the left-over blood in the umbilical cord and placenta (the organ that feeds the baby and comes out with the baby). A special program can store the cord blood of a full sibling for a patient with sickle cell disease.

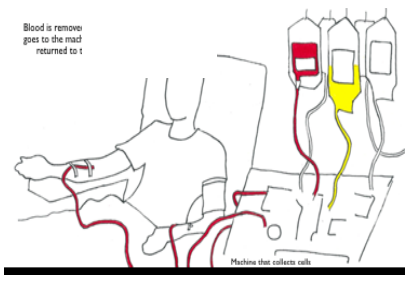
IS IT SAFE TO BE A DONOR?

- YES. But before someone can donate, a doctor first needs to check to make sure the donor is healthy. People with sickle cell trait can be BMT donors. There are some minor risks with donating bone marrow or peripheral blood stem cells. Donors may feel some pain, but usually this pain is mild and only lasts for a short time.

MARROW
Bone marrow is collected with a needle inserted into the hip bone.



BLOOD
Blood is removed from donor, goes to the machine, and then returned to the donor.



RESOURCES

sickle cell community consortium

info@sicklecellconsortium.org
Sicklecellconsortium.org

BE THE MATCH®

1 (888) 999-6743
patientinfo@nmdp.org
BeTheMatch.org/SickleCell

Sickle Cell Transplant Advocacy
& Research Alliance
Curesicklenow.org

Recruiting, US-based Trials for SCD
JCCTP.org/sickle-cell

For more information about the BMT for sickle cell disease, Please check out the following resources.

Talk to your doctor to learn more about BMT for sickle cell disease and you or your child's treatment options.

NOTES

NOTES

BLOOD MARROW TRANSPLANT (BMT) FOR SICKLE CELL DISEASE