

BLOOD AND MARROW TRANSPLANT (BMT) FOR SICKLE CELL DISEASE



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

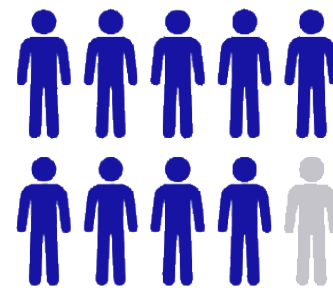
THIS HANDBOOK WAS DEVELOPED TO ANSWER COMMON QUESTIONS ABOUT BMT. PLEASE SHARE ANY QUESTIONS YOU MAY HAVE AFTER REVIEWING THIS BROCHURE WITH YOUR HEALTH CARE TEAM.

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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 age 16 years) and patients transplanted more recently (after 2007) had better outcomes.



On average when 10 children underwent matched sibling BMT, 9 of these children were cured.



IS BMT FOR ME OR MY CHILD



In the past, only children who had major problems 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 good 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**.



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.



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

It can be very difficult to decide to have or not have BMT. It is important to talk with your health care team and your family about your or your child's 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 stopped or slows down. For example, for most children the spleen can filter the blood better after BMT.

More organ damage is prevented. But permanent damage to organs like the brain after a stroke is not reversed after BMT.

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 should occur.

- **INFECTION**

After BMT, the immune system is weak so a patient can get serious infections. A patient needs to take medicines and follow special instructions to lower the chances of getting an infection.

- **REJECTION**

The patient can reject the new donor cells. If this happens, sickle cell disease returns.

- **LONG-TERM SIDE EFFECTS**

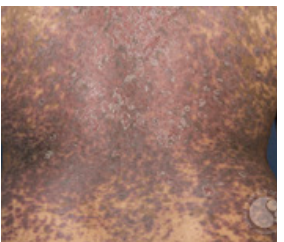
Chemotherapy given during preparation for BMT can cause infertility (Inability to have children), and may cause effects on other organs (heart, lungs, liver, kidneys, etc). If less chemotherapy is given, then these side effects may be less likely to occur. We are still learning about the long-term side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

- **GRAFT VS HOST DISEASE**

GVHD is a problem that can occur after BMT when the new immune system from the donor sees the patient's body as different and attacks it. 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**

Patients have died from problems after BMT, usually due to infection or GVHD.



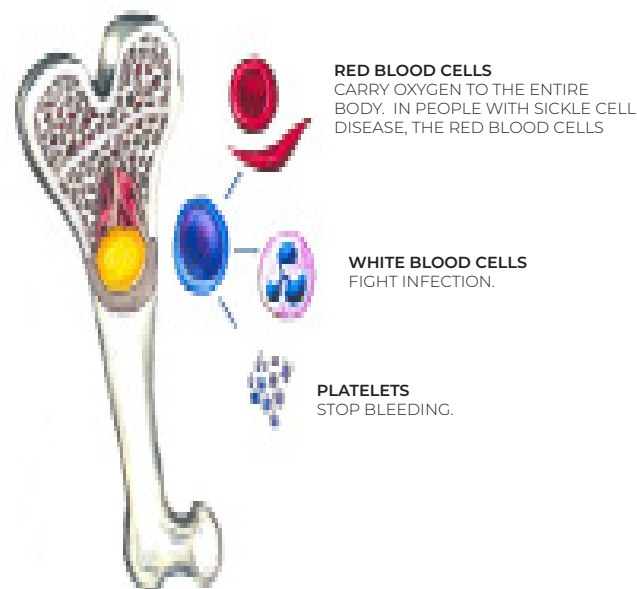
BMT BASICS

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 IN THE CENTER SPACE INSIDE BONES. THE BONE MARROW CONTAIN BLOOD STEM CELLS WHICH ARE SPECIAL CELLS THAT GROW TO BECOME RED BLOOD CELLS, WHITE BLOOD CELLS OR PLATELETS.



FINDING A DONOR: HLA TYPING

The first step for a patient thinking about BMT is looking for a donor. 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 like a blood type for your immune system. A BMT works best if a donor is an HLA-match.

How is HLA typing done?

A tube of blood or mouth swab is sent for testing. We recommend testing all healthy full siblings and parents. It usually takes a few weeks to get the results of this test.

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 will 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.

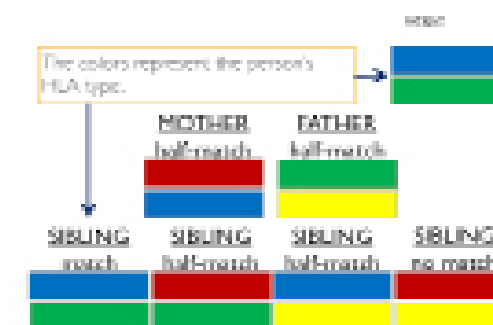
Parents usually are not a match for their children.

What if there is no matched sibling?

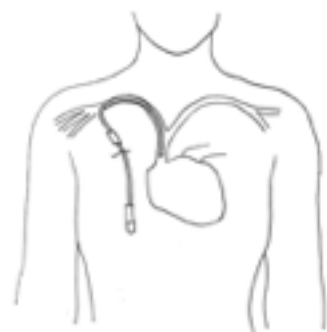
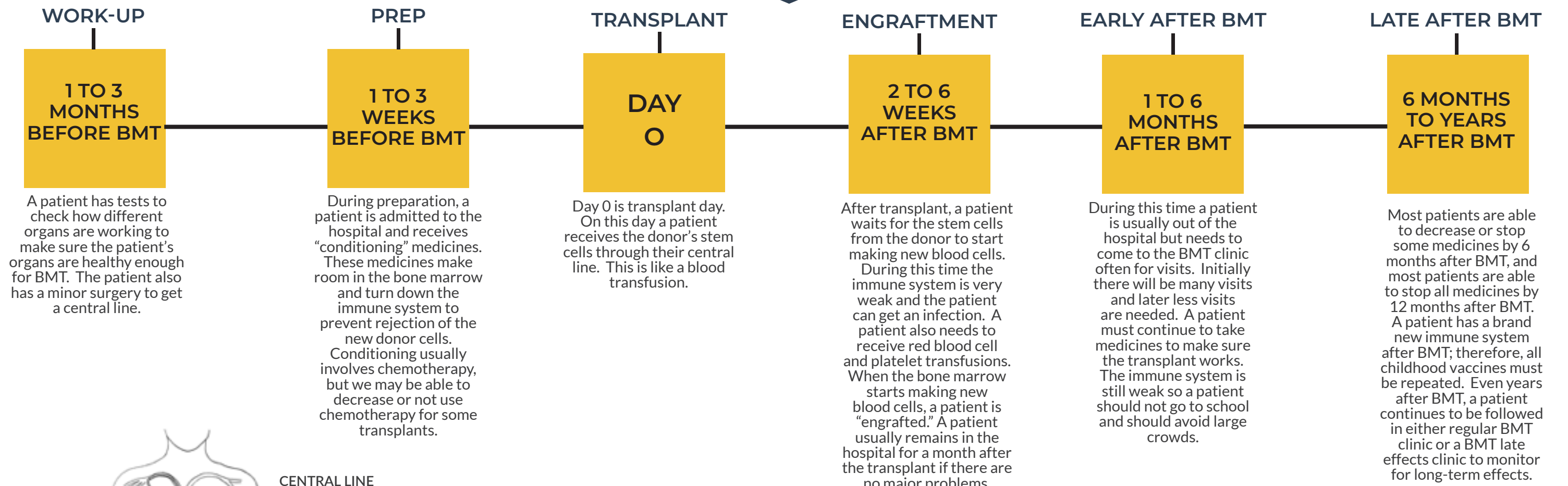
It is possible to use an "alternative donor." Alternative donors include:

- 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 TIMELINE



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.

BMT is a long process that involves six stages

BEING A DONOR

BONE MARROW

- Bone marrow inside the hip bone can be 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

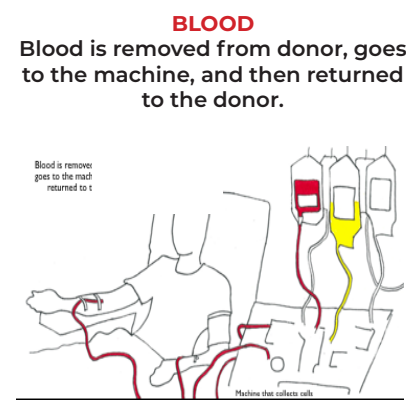
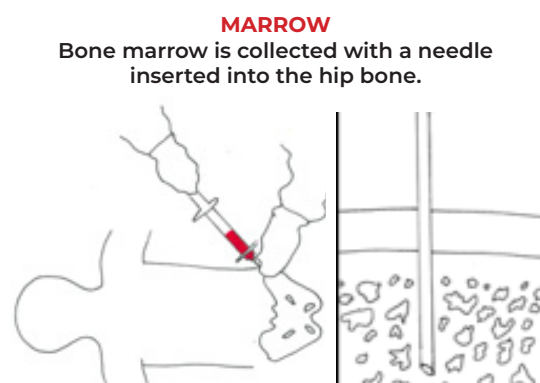
- A donor can be given a shot that contains a medicine called G-CSF. This medicine makes the stem cells leave the bone marrow and go out into the blood. This shot is usually given daily for 5 days. The stem cells are then collected with a special machine through a type of IV line.

UMBILICAL CORD

- After a baby is born, blood can be collected from the leftover 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.



RESOURCES



(706) 619-6029
info@sicklecellconsortium.org
Sicklecellconsortium.org



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.

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(BMT) FOR SICKLE CELL DISEASE**
