

Sickle cell disease: blood and marrow transplant (BMT)



What is a blood and marrow transplant (BMT)?

- A BMT helps to restore blood stem cells that are damaged, missing or not working.
- It replaces unhealthy sickle cells with healthy blood cells.
- It is the only cure for sickle cell disease at this time.
- BMT is not a surgery. It is a medical treatment that takes place over many months.
- Most often, at least the first month is spent in the hospital.

What is bone marrow?

Bone marrow is the soft, spongy tissue found inside the bones. It makes all the blood cells in your child's body. The cells in bone marrow are called blood forming stem cells. These stem cells form 3 major types of cells that the body uses. They are:

- White blood cells that fight infection
- Red blood cells that carry oxygen
- Platelets that help the blood to clot

How does a BMT help children with sickle cell disease?

BMT can be a cure for sickle cell disease when the normal red blood cells from a healthy donor replace the sickle red blood cells in your child.

What are the risks of a BMT for children with sickle cell disease?

A BMT is the only cure for sickle cell disease at this time, but there are risks. These risks include:

- Infection
- Low blood counts, which can lead to anemia
- Bleeding, stroke and other problems
- A weakened immune system for some time
- Rejection of the transplant
- Graft-versus-host disease [when the donor's cells (graft) attack your child's body (host)]
- Infertility (not being able to get pregnant or father a child)
- Death

A BMT can also put a lot of stress on patients and their families because it is such a long process. Your child will need:

- Antibiotics to prevent or treat infection
- Platelet transfusions to prevent or treat bleeding that may happen

In case of an urgent concern or emergency, call 911 or go to the nearest emergency department right away.

Sickle cell disease: blood and marrow transplant, continued

- Blood transfusions to treat anemia
- Medicines that lower the immune system for several months

What is the process for having a BMT?

1. Finding a donor

The best donor is a full sibling (brother or sister) who:

- Does not have sickle cell disease.
- Has the same “tissue type” as your child.

Another name for “tissue type” is HLA. HLA stands for “human leukocyte antigens,” which are proteins found on the surface of the cells in the body.

- Each full sibling has only a 1 in 4 (25%) chance of having the same HLA type.
- Half siblings would not make a good donor because they do not have the same HLA type.
- The donor does not need to have your child’s blood type but must have the same HLA or transplant tissue type.
- The donor **can** have sickle cell **trait** and still donate.

2. Preparing for transplant (treatment plan)

- Your child’s treatment plan is based on their condition.
- First, your child will get chemotherapy (chemo) and other medicines that make their immune system weak.
- The medicines get rid of your child’s old bone marrow cells. They also prepare the body to get the new, healthy blood stem cells from the donor.
- The medicines may cause side effects, such as nausea, vomiting (throwing up), hair loss, mouth sores, poor appetite, diarrhea and low blood counts. These side effects are temporary.

3. The BMT

- During the actual BMT process, blood stem cells are taken from the donor.
- Your child gets the blood stem cells from the donor through an intravenous line (I.V. line).
- It is like getting a blood transfusion.
- It can take a few minutes to a few hours.

4. After the transplant

- The new blood stem cells travel to your child’s bone marrow.
- It may take up to 2 to 4 weeks for your child’s body to produce new healthy cells.
- Your child could stay in the hospital for 4 to 6 weeks.
- After leaving the hospital, your child may take several medicines for many months.
- Your child will make many visits to the BMT clinic as they recover.
- Your child will have to stay out of school and follow special infection rules for a time.

In case of an urgent concern or emergency, call 911 or go to the nearest emergency department right away.

Sickle cell disease: blood and marrow transplant, continued

Can my child get a BMT?

Due to the serious risks, **at this time** a BMT is offered to younger patients with more severe sickle cell disease. These patients are at the highest risk for disability or death.

The most common reasons for a BMT are:

- Stroke or a high risk for stroke
- Frequent acute chest syndrome
- Frequent pain crises

Many children with severe sickle cell disease are not able to have a BMT because they do not have a healthy matched-sibling donor. For them, the risks of a BMT are higher, so a transplant is an option only in rare cases.

What should I do if my child is sick?

- Follow the sickle cell provider's advice for what you should do.
- Call your child's provider anytime your child has a fever of 101°F (38.3°C) or higher and is sick. Your child will need to be seen **right away**.
- During the time when the clinic is closed (between 5 p.m. and 8 a.m. during the week and on weekends and holidays), call the hematologist on call. They will contact the emergency department (ED) and help with your child's care.
- **ALWAYS** tell the ED doctors and staff that your child has sickle cell disease. Also tell them which sickle cell clinic your child visits.

For more details on sickle cell disease and services, visit the Children's Healthcare of Atlanta website at choa.org/sicklecell.

This teaching sheet contains general information only. Talk with your child's doctor or a member of your child's healthcare team about specific care of your child.

In case of an urgent concern or emergency, call 911 or go to the nearest emergency department right away.