

Bone Marrow (Stem Cell) Transplant for Sickle Cell Disease



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This document is not intended to take the place of the care and attention of your personal physician. Our goal is to promote active participation in your care and treatment by providing information and education. Questions about individual health concerns or specific treatment options should be discussed with your physician. For more general information on sickle cell disease, please visit our Web site at www.stjude.org/sicklecell.

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How did bone marrow (stem cell) transplants begin for children with sickle cell disease?

Bone marrow (stem cell) transplants have been used for the treatment and cure of a variety of cancers, immune system diseases, and blood diseases for many years.

Doctors in the United States and other countries have developed studies to treat children who have severe sickle cell disease with bone marrow (stem cell) transplants.

How does a bone marrow (stem cell) transplant work?

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In a person with sickle cell disease, the bone marrow produces red blood cells that contain hemoglobin S. This leads to the complications of sickle cell disease.

- To prepare for a bone marrow (stem cell) transplant, strong medicines, called chemotherapy, are used to weaken or destroy the patient's own bone marrow, stem cells, and infection fighting system.
 - This is done so the patient does not reject the new blood cells coming from the donor.
- The patient's bone marrow then is replaced with blood-forming stem cells from a donor who does not have sickle cell disease.
 - This can be a donor with normal hemoglobin or sickle cell trait.
 - The actual transplant is given like a blood transfusion through an IV tube.

- The new bone marrow then produces red blood cells that are healthy since they do not contain a lot of hemoglobin S.

Who can donate stem cells for transplants?

There are 3 main types of stem cell donors:

- **Matched related** – A brother or sister who has the same bone marrow type and the same mother and father.
 - Brothers and sisters are matched through special blood tests called HLA typing.
- **Matched Unrelated** – volunteers who have the same bone marrow type as the patient
 - Usually these types of donations are matched through national organizations that match donors and patients who have the same type of bone marrow.
- **Haploidentical** – half-matched family members (usually a mother or father)
 - This type of donation is still considered experimental and should be performed only as part of a research study.

Stem cells can be obtained from the donor's bone marrow or peripheral blood (blood in the veins). Or in some cases, stem cells are collected from the umbilical cord at the time of birth.

What are the benefits of bone marrow (stem cell) transplant?

Bone marrow (stem cell) transplant is the only treatment available today that can cure sickle cell disease. If the transplant is successful, the patient is cured from sickle cell disease.

- St. Jude Children's Research Hospital was the first organization to find the cure for sickle cell disease through bone marrow transplant.
- In 1982, a St. Jude patient had leukemia and sickle cell disease. A St. Jude doctor performed the transplant using bone marrow donated from the patient's brother. The patient was cured of both leukemia and sickle cell disease.
- The doctor wrote about this case in *The New England Journal of Medicine* and presented it at the 25th Annual Meeting of the American Society of Hematology so other people could benefit from the cure.

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What are the possible risks of transplant?

- **Infections** – Chemotherapy lowers the white blood cells, which normally fight and prevent infections.
 - This puts the patient at high risk for infections, which can be caused by bacteria, fungi, or viruses.
 - Medicines are given to fight these germs and prevent these infections. Infections that do not respond to the treatment can lead to death in 5 to 10 percent of patients.

- **Graft-versus-host disease (GVHD)** – This reaction occurs when the immune cells of the donor (graft cells) sense that cells of the patient (host cells) are different and attack them. This can be a serious side effect of transplant.
 - GVHD occurs in up to 10 percent of patients who undergo matched related types of transplants. It can be higher in transplants using other donors.
 - This condition can be acute (occurring less than 100 days after the transplant) or chronic (occurring more than 100 days after transplant).
 - It may cause damage to the skin, liver, and intestinal tract of the transplanted patient.
 - Drugs are given to prevent or limit GVHD. These drugs increase the patient’s risk of infection. However, GVHD that does not respond to treatment can lead to organ damage or even death.
- **Graft failure** – There is about a 10 percent risk that the new bone marrow from a matched family member will fail to take. The chance of graft failure is higher with other types of donors.
 - This means that the patient will not be able to make any white blood cells, red blood cells, or platelets. Typically, the transplant would need to be repeated.
 - If there are no more donor cells, stem cells collected from the patient before the transplant can be given back to the patient. This restores the patient’s original bone marrow, which means the sickle cell disease comes back.
- **Veno-occlusive disease (VOD)** – Blood vessels that lead into and pass through the liver may suffer damage after a bone marrow transplant. This may be caused by the chemotherapy and might lead to swelling and severe liver damage.
 - The chances of severe VOD are about 1 out of every 20 cases.

- **Nutrition problems** – The stomach and intestines are sensitive to chemotherapy. Nausea, vomiting, mouth sores, diarrhea, and loss of appetite may occur.
 - Typically, nutrition must be given through the veins until patients are able to eat.
- **Low blood counts** – While waiting for new stem cells to make normal red blood cells as well as all other types of cells, a patient usually needs transfusions of platelets and red blood cells.
- **Social and emotional concerns** – A bone marrow transplant is challenging to both the patient and family members. The patient’s routine may change for a while because he will be away from home, isolated from school, friends, and relatives.
 - The family is the key to supporting the child through the transplant period.
 - The average time spent in the hospital is 4 to 6 weeks. Patients who live out of town will need to remain near St. Jude for 100 days or longer, depending on their health after transplant.
- **Infertility** – Most patients who receive a transplant will not be able to have their own children in the future. This is one (1) possible side effect of drugs used while preparing for the transplant; however, there have been patients who were able to conceive children after having a transplant.

What is required for a transplant?

Two major requirements must be met for a transplant to proceed.

1. The first is to identify the person who is the best donor. Blood tests will determine who the best match is.
2. Secondly, after the best donor is chosen, both the donor and the patient will have pre-transplant evaluations of the heart, lungs, kidney, etc.

Interviews with a psychologist and a social worker also are an important part of the process.

Is bone marrow (stem cell) transplant the only treatment for sickle cell disease?

It is the only cure for sickle cell disease at this time. Results of many studies show that transplants from matched related donors offer about an 85 percent chance of cure. Other treatment choices are the drug hydroxyurea and chronic red blood cell transfusions. These treatments may lessen the complications of sickle cell disease, but they do not cure the disease.

Who can I talk to about a bone marrow (stem cell) transplant?

At St. Jude Children's Research Hospital, we have experience in treating children with sickle cell disease with bone marrow (stem cell) transplant. Our doctors and other health care experts can answer your questions. We encourage families to discuss treatment options and to ask questions to learn more about the care of their children with sickle cell disease.

Notes



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