

Transplant for sickle cell disease

What is a blood stem cell transplant?

A blood stem cell transplant is a treatment that can cure sickle cell disease. It replaces your unhealthy blood-forming cells with healthy cells from a donor, and is not surgery. It is more like a blood transfusion. It cannot fix organ damage from sickle cell disease (SCD), but it can stop more damage from happening. Transplant is a journey. The goal is to help you live free from sickle cell pain, prevent any new complications, and improve your overall quality of life.

How does a blood stem cell transplant work?

The blood stem cell transplant journey includes the major steps listed below but everyone's journey will look a little different. The first step is finding a donor that matches your human leukocyte antigen (HLA) type. Doctors match patients and donors by looking at your HLA, which is a protein-or marker-found on most cells in your body. It helps your immune system recognize your own cells as normal and things like infections as not normal. Your health care team will give you information and support every step.

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1 Your doctor looks for a donor who matches your HLA type. You and potential donors will have blood drawn or the inside of your cheek swabbed to tell us your HLA type.
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2 Once a donor is found, the process begins. The donor could be a family member, like a brother or sister, or someone unrelated to you. You can get donated cells from someone even if they have sickle cell trait!
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3 Your doctor will do many tests to make sure that your body is ready for the transplant.
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4 You will get chemotherapy and maybe radiation to destroy your unhealthy blood-forming cells. Destroying the unhealthy cells makes room for the donor's healthy cells.
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5 Your donor's blood-forming cells are collected from their bone marrow or blood and prepared for you.
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6 You get the donor's cells through an intravenous (IV) catheter, or tube, like a blood transfusion.
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7 The donor's cells find their way into your bone marrow to make healthy white blood cells, platelets and red blood cells that don't sickle.
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8 Your transplant team will watch you closely to see how the new cells are working and to look for signs of infections and other problems. Most people are hospitalized for at least a few weeks and possibly longer.

Who can get a blood stem cell transplant?

Talk with your doctor about whether transplant is a treatment option for you. Doctors might recommend transplant if you have had:

- 8 or more red blood cell transfusions in a year
- Chronic pain lasting longer than 6 months
- More than 3 severe pain crises in the last 2 years
- High blood pressure in your lungs and heart (pulmonary hypertension)
- Acute chest syndrome 2 or more times in the last 2 years
- Tests that show you may be more likely to have a stroke
- A stroke or silent stroke

Even if you have not had any of the above, transplant may still be an option for you if you have a brother or sister with your same HLA type. Talk to your doctor about HLA testing to see if there is a match in your family.

What are the risks and side effects of transplant?

Transplant does come with risks. Every person will have a different experience. Your experience may depend on your age, the type of donor you use, your health before the transplant and other factors. Some side effects can happen early after transplant, but others can happen sometimes years after transplant.

Some risks of transplant include:

- Your body rejecting the new cells (graft failure)
- Graft-versus-host disease (GVHD), when the donated cells fight your body
- Infections
- Bleeding
- Seizures
- Not being able to have kids (infertility)

Sometimes there are significant complications that can result in death.

It is important to talk to a transplant doctor about your risk, potential side effects and complications. You, your family and care team can weigh the risks and benefits together to decide if transplant is right for you.



Spencer, blood stem cell recipient, with his son.

Questions to ask your doctor

Your hematologist or primary care doctor may be able to give you more information and refer you to a transplant doctor. A transplant doctor can talk about all your options. Here are a few questions to get the conversation started.

- What are the chances that transplant will cure my SCD?
- What are the risks of waiting or trying other treatments before transplant?
- Will I lose my hair with transplant?
- How does transplant affect my ability to have kids? Are there resources to help me have kids after transplant?
- What would my next steps be if I want to learn more about transplant?
- Are there other curative options available for me?

Please ask questions! It's important that you learn about your options before making treatment decisions.

Resources for patients with sickle cell disease

We offer free support and resources for you and your loved ones to help you navigate life with sickle cell disease and to learn about your treatment options. Visit sicklecellconnect.com.

For more information:

Call: 1 (800) 999-6743 Email: patientinfo@nmdp.org

