

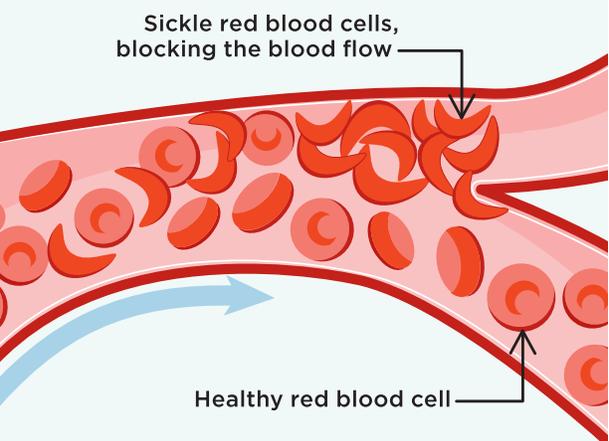
What is Sickle Cell Disease?

Sickle cell disease (SCD)

is an inherited blood disorder that causes a protein in your blood, called hemoglobin, to be misshapen.

The misshapen, or sickled, blood cells can get stuck in your blood vessels, which damages your organs and tissues.

When your blood vessels get blocked by the sickled blood cells, you can have severe pain, infection, strokes and other serious health problems.

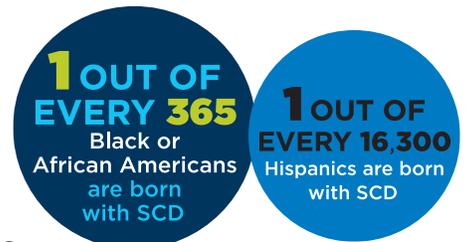


HOW MANY PEOPLE ARE AFFECTED?

SCD affects over 100,000 people in the U.S. Symptoms and complications can be mild to severe. For most people, the disease gets worse over time.

WHO DOES IT AFFECT?

In the U.S., SCD is more common among Black or African Americans



HOW CAN IT BE TREATED?

SCD affects each person differently, so there is no “best treatment” for everyone with SCD. **There are several medicines** approved by the U.S. Food and Drug Administration (FDA) to help lower the amount of pain crises or SCD complications people experience. They include:

- Crizanlizumab
- Hydroxyurea
- L-glutamine
- Voxelotor

Other options to reduce SCD symptoms include:

- Pain medicine
- Blood transfusions
- Medicines to prevent blood clots
- Antibiotics

IS THERE A CURE?

A blood or marrow transplant (BMT) is the only known cure for SCD. BMT is a treatment that uses healthy blood-forming cells donated by someone else to replace your unhealthy blood-forming cells. The cells are given to you through an intravenous (IV) infusion.

WHAT OTHER TREATMENTS ARE AVAILABLE?

Doctors and researchers are studying new treatments for SCD. One possible cure is called gene therapy. Right now, gene therapy is only available through a clinical trial. Learn more about gene therapy and other treatments for SCD at [JCCTF.org](https://www.jcctf.org).

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 learn about your treatment options.

Call **1 (800) 999-6743** or email patientinfo@nmdp.org



SUPPORT & GUIDANCE

Not sure where to go or who to ask? We'll listen and help you find answers. **We can help you:**

- Navigate the health care system
- Learn about blood or marrow transplant (BMT) as a treatment option
- Get answers to your financial or insurance questions



EMOTIONAL SUPPORT

We provide confidential, one-on-one telephone-based counseling. We can help you and your loved ones talk through emotional, social, or practical things related to living with sickle cell disease.



FINANCIAL ASSISTANCE

Grants are available for qualified patients to pay for prescriptions, housing, food, gas and more.



PEER CONNECT

We can put you in touch with a trained volunteer who's been there. People who've received a BMT for their sickle cell disease are available to talk by phone or email, sharing their experience and tips.



TREATMENT OPTIONS

We can help you and your family learn about clinical trials as potential treatment options. We offer:

- One-on-one guidance from a clinical trial navigator who can answer questions and help you search for clinical trials
- An easy-to-use online tool to search for clinical trials and alert you when new trials begin
- Educational resources to learn about research, clinical trials and new treatments

Transplant for Sickle Cell Disease

WHAT IS A BLOOD OR MARROW TRANSPLANT (BMT)?

- It's a treatment that uses healthy blood-forming cells from a donor to replace your abnormal cells. It is **not** surgery.
- It's the only known cure for sickle cell disease (SCD).
- It can't reverse organ damage from SCD but it can stop more damage from happening.

HOW DOES BMT WORK?

- First, you'll get chemotherapy, and maybe radiation, to destroy your abnormal blood-forming cells.
- Then, the donor's blood-forming cells are given to you through an intravenous (IV) catheter, or tube, like a blood transfusion.
- From there, the cells find their way into your bone marrow to make healthy red blood cells, white blood cells and platelets.

The entire process, starting with treatment through recovery, can take months to years.

WHERE DO THE NEW CELLS COME FROM?

The donor could be a family member or someone unrelated to you. You can get donated cells from someone even if they have sickle cell trait.

Doctors match patients and donors by looking at human leukocyte antigen (HLA) markers on their cells. Your doctor will first look for a matching donor in your family. Biological siblings have a 1 in 4 chance of being a full match for each other. Biological

parents and children are always a half-match for each other (this is called a haploidentical transplant). If you don't have a matched donor in your family, your doctor can search the Be The Match Registry®.

WHO CAN GET A TRANSPLANT?

People with severe SCD. This includes people who've had a stroke, organ damage or frequent pain crises. Transplant has worked very well for children who have a matched sibling donor.

Many patients get a transplant by joining a clinical trial. Our clinical trial navigators can help you explore your treatment options. Learn more at JCCTP.org.

WHAT ARE THE RISKS AND SIDE EFFECTS?

Although transplant can cure SCD, it has risks, including your body rejecting the new cells, infections, infertility, temporary hair loss, graft-versus-host disease (GVHD) and possibly death. Talk to a transplant doctor to learn more about your risks.



Desiree, transplant recipient for SCD, with her doctor

QUESTIONS TO ASK YOUR DOCTOR

- Is transplant an option for me?
- What are the chances that transplant will cure the SCD?
- What are the risks of waiting or trying other treatments before transplant?
- Does my age affect the risks of transplant?
- What are the possible side effects of transplant? How can they be reduced?
- How might my quality of life change over time, with or without transplant?

You could be the cure for sickle cell



Tinisha, (above, right) donated to cure Zalika (left) from sickle cell disease.

Registry members **like you** could be the life-saving match for patients in need like, Thor (pictured below). He is still in need of a marrow match to cure his sickle cell disease.



A BLOOD OR MARROW TRANSPLANT IS THE ONLY KNOWN CURE FOR SICKLE CELL DISEASE (SCD)

- 1 out of every 365 Black or African Americans in the U.S. are born with sickle cell disease.
- Patients are most likely to match someone of the same ethnic background.
- Only 23% of Black or African American patients are likely to find the marrow match needed for their cure.

HOW TO SAVE A LIFE



1. Register Online

We'll ask you a series of questions to see if you meet basic qualifications.



2. Swab Cheek

It's easy—we will mail you a kit to swab your cheek for a sample of your DNA.



3. Get Matched

Genetics are complex. It could be weeks, months or years before you are contacted to donate.



4. Donate

Be ready, if called to donate you're the patient's best genetic match from the entire registry and perhaps their only hope for a cure.