Why Participate in Clinical Trials for Sickle Cell Disease?

Clinical trials are an important step in finding new and improved ways to identify and treat diseases. If you take part in a clinical trial, you may receive new treatments before they are widely available. By participating in a clinical trial for sickle cell disease (SCD), you could help researchers to better understand the disease. You could also help researchers find new treatments that can be given to SCD patients in the future.

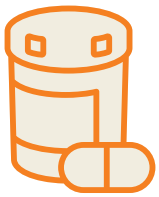
ASH RESEARCH COLLABORATIVE®
Accelerating Progress in Hematology

Patient Safety in Clinical Trials

Patient safety and well-being are a top priority during a clinical trial. In fact, before a new treatment is given to patients in a clinical trial, it must go through a testing process. A new treatment is also called an "investigational treatment."

- The investigational treatment is first studied in a lab to find out how safe it is and how well it works in animals and human cells before human use.
- The clinical trial is reviewed by the U.S. Food and Drug Administration (FDA) to ensure that there are guidelines in place to protect the participants.
- The clinical trial is also reviewed by an independent group of doctors, researchers, and members of the community called an institutional review board (IRB). The role of the IRB is to make sure that the trial is fair and that the rights and welfare of participants are protected.

40+
treatments in development for SCD

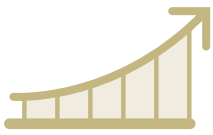


May get access to new treatments.

The medical community is working hard and researching new ways to treat sickle cell disease. During a clinical trial, the investigational treatment is provided at no cost to you.

Receive medical care.

Some clinical trials offer free medical care. The best way to find out if a clinical trial is an option for you is to speak with your doctor.



Help advance the treatment of sickle cell disease.

You can help advance the treatment of sickle cell disease by participating in a clinical trial. Participants in past SCD trials helped drive today's treatments for the disease. Your participation could help future SCD patients live longer, healthier, happier lives.

What to Expect When You Participate in a Clinical Trial for Sickle Cell Disease

Before the trial

The trial doctor will get to know you and your medical history to make sure that the trial is right for you. You'll have an open discussion about your condition and lifestyle, and about any questions or concerns you may have.

The FDA requires that all medicines are tested for safety and shown to be effective before they are approved and available on the market. Even once a treatment is approved, the FDA keeps track of its safety.

	Phase I	Phase II	Phase III	Phase IV
Typical Length	a few months	3-12 months	6-18 months	1 year to many years
Number of Patients	10-20	50-75	100-300	100-300
Purpose	Study safety, side effects, and dosage of treatment	Learn about treatment effectiveness and further study the safety of treatment	Confirm effectiveness and monitor safety while comparing to other treatments and collecting more information	Provide additional information after approval, including risks, benefits, and best use

During the trial

During a clinical trial, researchers often compare the investigational treatment to the best treatment that is already available for patients. They study the safety of the new treatment, and they study its effect on disease symptoms.

Phase Overview

Before any drug or treatment becomes available to the public, clinical trials are necessary to test its safety and show that it is effective. Even after the FDA approves the drug, additional trials allow the FDA to keep monitoring the drug's safety and effectiveness.

The graphic below shows the different phases of clinical research. It is important to note that the "typical length" refers to the amount of time each participant is usually in the trial, not the entire length of the trial. The length of the trial is often longer because participants start at different times, and the researchers must study the results after the trial is over.

Randomization

Clinical trial randomization is the process of assigning patients by chance to groups that receive different treatments. In the simplest trial design, the investigational group receives the new treatment and the control group receives standard therapy or placebo. At several points during and at the end of the clinical trial, researchers compare the groups to see which treatment is more effective or has fewer side effects.

Use of Placebo in Clinical Trials

If participating in a clinical trial, you may be randomized to a group receiving a placebo. Those who get placebos in clinical trials serve an important role. Their responses help provide a good way to measure the actual effect of the treatment being tested. The placebo group provides an important baseline used to compare the treatment group.

After the trial

Researchers will study the data collected during the trial and publish the results. Depending on the type of trial, this process can take one to two years. Participants can follow the trial's progress online or with the clinical trial care team to learn about results.

At the end of the clinical trial, the researchers decide if more information is needed. If so, then more trials will be done. In some cases, participants can continue to receive the investigational treatment, if it is working well for them.

No matter what the outcome of the trial is, your participation will have helped researchers learn more about sickle cell disease. Everything that they learn helps them to develop future treatments and improve the quality of life for future patients.

How can I get involved?

To learn more about clinical trials and find opportunities to participate in your area, please visit *www.ashresearchcollaborative.org/contact-us*.

ASH RESEARCH COLLABORATIVE®

Accelerating Progress in Hematology

The ASH Research Collaborative (ASH RC)® was created by the American Society of Hematology to encourage partnerships that help progress hematology research. Its goal is to improve the lives of people affected by blood diseases.

The ASH RC has created the Sickle Cell Disease Research Network as part of a broader effort focused on SCD patient education and engagement. The mission of the network is to improve outcomes for individuals with sickle cell disease by increasing the number of effective treatment options.