



National Heart, Lung, and Blood Institute (NHLBI)

Participation in Clinical Research
on Sickle Cell Disease (SCD)

The only way to know for certain whether a new treatment will help or harm people with a particular condition is to conduct a *clinical trial*. This is a formal way of looking at treatments to determine whether and how well they work, for whom they are appropriate, and how many side effects they cause. The NHLBI has been the home for SCD clinical research since the late 1960s, and has conducted a number of landmark trials that demonstrated the effectiveness of:

- § penicillin begun soon after birth to prevent life-threatening infections
- § transcranial Doppler to detect children at high risk of stroke
- § blood transfusions to prevent stroke
- § hydroxyurea to reduce pain crises, acute chest syndrome, the need for blood transfusions, and deaths.

Clinical research is complex and expensive. It requires a dedicated and coordinated effort not only by the patients and their families who volunteer to be part of the studies, but also by a host of others including the following:

- § *Investigators* who design and conduct studies in people are scientists, many of whom are physicians, as well as experts in other fields such as statistics. Many are on the faculty of universities or in community practices where they see patients with conditions under study. Investigators come up with most of the ideas for clinical studies, and their passion to advance the science and improve the lives of their patients is essential.
- § *Sponsors* who support clinical trials can come from industry, private foundations, or the federal government. The federal National Institutes of Health (NIH), of which the NHLBI is a part, conducts and supports research with the goal of improving the health of individuals and the public as a whole. Taxpayer money is appropriated by the U.S. Congress for this purpose.
- § *Data and Safety Monitoring Boards* are composed of scientists, ethicists, and patient representatives who oversee a study as it is being conducted to safeguard the health and well-being of participants. They are independent of the investigators and the sponsors of the study to ensure an unbiased focus on the welfare of the patients who participate.

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The NHLBI, in partnership with other NIH components and the National Marfan Foundation, Institutes, recently unveiled a comprehensive Web site, [Children in Clinical Studies](#), to give parents and health-care providers the information they need to understand clinical research in children and make informed decisions about participating in a study. It includes a video that may be useful for adults thinking about participating in a clinical trial as well.

A database of all open clinical trials, whether they are sponsored by the NIH or by some other entity, is available at <http://clinicaltrials.gov>. You can search the site for studies of any condition, including SCD, and find out whom to contact if you are interested in participating. The site also includes studies that are no longer enrolling participants so that you can find out about clinical trials that have recently been completed.

Examples of NIH-sponsored trials in SCD that are currently recruiting participants are provided below.

Stroke with Transfusions Changing to Hydroxyurea (SWITCH)

Issue: Children with SCD who have experienced a stroke receive monthly blood transfusions to prevent them from having another stroke. Although chronic transfusions are effective, they are risky. Moreover, children who receive them develop iron overload, which much be treated with an inconvenient and painful therapy called chelation. Is there a better approach to prevent secondary strokes and remove excess iron from the bloodstream?

Participants: Children with SCD, 5 to 18 years of age, who have been treated with blood transfusions for at least 18 months since having a stroke

Comparison: hydroxyurea + phlebotomy (removal of blood) (the experimental treatment)
versus

blood transfusions + chelation (the standard approach)

More Information: <http://www.clinicaltrials.gov/ct2/show/NCT00122980>

Silent Cerebral Infarct Multi-Center Clinical Trial

Issue: Silent cerebral infarcts/strokes that do not cause immediately obvious symptoms frequently go unrecognized but are one of the most serious complications of SCD. They can cause declines in school performance, increased forgetfulness, and a diminished ability to follow even simple instructions. Are blood transfusions effective and safe in preventing them in high-risk children?

Participants: Children with SCD, 5 to 14 years of age, who have had a cerebral infarct documented by MRI scan

Comparison: blood transfusions (the experimental treatment)
versus

no intervention (the control)

More Information: <http://www.clinicaltrials.gov/ct2/show/NCT00072761>

Examining Cognitive Function and Brain Abnormalities in Adults with Sickle Cell Disease

Issue: Adults with SCD may have brain abnormalities that contribute to problems with cognitive functioning, including attention and memory difficulties. Can these problems be prevented by chronic blood transfusions?

Participants: Adults with SCD, 21 to 55 years of age

Comparison: blood transfusions (the experimental treatment)
versus
no intervention (the control)

More information: <http://www.clinicaltrials.gov/ct2/show/NCT00528801>

Sildenafil therapy for Pulmonary Hypertension and Sickle Cell Disease

Issue: Pulmonary hypertension, a common complication of SCD, heralds a serious risk of early death in affected patients. Can treatment with sildenafil improve functioning and increase survival?

Participants: Persons with SCD, 12 years of age or older, who have at least mild pulmonary hypertension

Comparison: sildenafil (the experimental treatment)
versus
placebo (the control)

More information: <http://www.clinicaltrials.gov/ct2/show/NCT00492531>

Stem Cell Transplant in Sickle Cell Disease and Thalassemia

Issue: Bone marrow transplantation has been used increasingly for the long-term treatment and cure of SCD and beta thalassemia. However, severe side effects can occur as a result of the high-intensity chemotherapy that is used to prepare the patient for transplant. Can use of lower-intensity chemotherapy result in successful bone marrow replacement with less-severe side effects but permanent control of the disease?

Participants: Patients with SCD or thalassemia, 1 month to 21 years of age, who are highly symptomatic

Comparison: transplant of stem cells, from either a family-related or cord-blood Bmatched donor, following moderate-intensity chemotherapy (the experimental treatment)
versus
(no control group)

More information: <http://www.clinicaltrials.gov/ct2/show/NCT00408447>

Evaluating the Safety and Effectiveness of Stem Cell Transplants from Unrelated Donors in Children with Sickle Cell Disease (The SCURT Study)

Issue: Bone marrow transplantation has been used increasingly for the long-term treatment and cure of SCD. However, side effects can occur as a result of the high doses of chemotherapy and other medications that are used to prepare the patient for transplant. Is a conditioning regimen that uses lower doses of chemotherapy and medications effective and safer?

Participants: Children with severe SCD, 3 to 16 years of age, who lack a sibling with the same tissue type to serve as their donor

Comparison: transplant of stem cells, using either bone marrow or cord blood from an unrelated donor, following a lower-dose conditioning regimen (the experimental treatment)

versus

(no control group)

More information: <http://www.clinicaltrials.gov/ct2/show/NCT00745420>

Hypnosis to Manage Pain and Symptoms in Patients with Sickle Cell Disease

Issue: Standard medical therapies for controlling painful crises in SCD patients are limited because of ineffectiveness and/or toxicity. Can a cognitive-behavioral intervention centered on self-hypnosis for pain management reduce pain frequency, improve sleep quality, and decrease use of narcotic pain medications?

Participants: Patients with SCD, 18 years of age or older, who have recently experienced significant problems with pain.

Comparison: weekly hypnosis for 4 weeks, followed by daily self-hypnosis (the experimental treatment)

versus

weekly health education for 4 weeks (the control)

More information: <http://www.clinicaltrials.gov/ct2/show/NCT00393250>