

Length of the SCURT study

Your child will be in the study for two years. Your child's doctor will keep the study doctors informed about your child's health. If you decide to change your child's doctor, please inform your child's study doctors so they can get all of the information they need for the study from your child's new doctor.

The risks of the SCURT study

Your child may have some possible side effects from the medications given in this study, such as:

Skin rash	Fevers	Diarrhea
Hair loss	Mouth sores	Vomiting
Nausea	Infections	Bleeding

These side effects are usually temporary.

Even with medications to prevent it, your child may get a condition called graft versus host disease (GVHD) from the donor cells. GVHD occurs when donor cells sense that the recipient's cells are different from them and attack them. This can cause skin rash, vomiting, diarrhea, and liver problems. These problems are usually mild, but can become very serious and life-threatening.

It is possible that the old red blood cells will grow back. If your child partially or fully rejects the donor cells, the severe sickle cell disease may persist or come back.

The complications of an unrelated donor transplant may be severe enough to cause death. Doctors will be sure to make the transplant as safe as possible for your child, but there is a 10% risk of death following this type of transplantation.

The cost of the SCURT study

Your insurance company will pay for the standard care, which is most of the care given in this study. The costs of standard care include staying in the hospital, doctor visits, standard lab tests, medications, and the cost of the donor's marrow. There is no charge for research tests. You or your child will not be paid to participate in this study.

Who will see your child's medical information

Your child's medical information will be shared with the study doctors and study research staff. The study doctors will do their best to keep your child's medical information private, but they cannot guarantee total privacy. Your child's medical information may be given out if required by law. If information from this study is published in a scientific journal or presented at scientific meetings, your child's name will not be used.

For Information

Speak with your doctor about the Study
or contact

The National Marrow Donor Program®
Office of Patient Advocacy
1-888-999-6743
E-mail: patientinfo@nmdp.org
Web site: marrow.org/patient

The SCURT study is supported by:



Unrelated Donor Bone Marrow Transplantation for Children with Severe Sickle Cell Disease

SCURT Clinical Trial: A Summary for Parents



The Blood and Marrow Transplant Clinical Trials Network (BMT CTN) develops clinical trials, also called research studies, about bone marrow and cord blood transplantation. The BMT CTN works to find the best transplant treatments and improve transplant results.

It is a good idea to talk about the information given in this summary with your child’s study doctor or nurse. They can help answer questions and give you more details about this study. Family and friends can also be a source of help to understand your choices. Having your child join a research study is an important decision, and we want you to have all the facts you need before you decide.

The goal of the SCURT study

Bone marrow is a developing treatment for sickle cell disease (SCD). The idea is to replace the cells in the bone marrow that produce sickle cells with cells from a healthy donor that will produce normal blood cells. In the past, most children with SCD who had a transplant received cells from a sister or brother who matched them (had the same tissue type). Most of them received high doses of a particular combination of drugs to make sure that the donor cells engraft (or take).

This study will look at whether a new combination of drugs to prepare patients to receive donor cells that uses a less intense combination of drugs is better than the standard preparation. It will also look at whether children with severe SCD who receive bone marrow from a person who is not related is effective in improving their SCD related symptoms. The results may help doctors learn more about whether this type of transplantation can treat or even cure SCD.

Why your child was invited to join this study

A clinical trial is an important way to answer a medical question. Your child’s participation in this study adds information to what doctors know about treatment choices for patients with severe SCD which may help patients live longer, healthier lives. It is important to know that your child may not benefit directly by joining the trial.

Who can join the SCURT study

To join this study, your child must:

- ◆ Be younger than 20 years old
- ◆ Have severe SCD, along with one or more of the following:
 - ✓ Stroke or other SCD brain complication
 - ✓ Severe chest pain more than once
 - ✓ Severe pain problems more than twice
 - ✓ Increased blood flow rates in the brain vessels as measured by a doppler
- ◆ Not have a healthy brother or sister as a matched donor
- ◆ Have an unrelated donor that is a suitable match and available
- ◆ Have signed permission to join the study from a parent or guardian
- ◆ Give his/her assent to join the study, if able

How your child can be in the SCURT study

It is your decision to have your child in the study. To learn if your child can be in the study, check with the information given under *Who can join this study* (above) and talk with the study doctor taking care of your child. If you decide to have your child join the study, you will need to give permission. If your child is old enough to understand the study and its risks and responsibilities, he or she will also need to give permission.

The study plan

Before your child begins the study, he or she will go through tests to determine if he or she can be in the study. Not everyone who is interested will be able to join.

To prepare for transplantation, your child will have tests and meet the transplant team before the study starts. Your child will receive medications to prepare the body for the new bone marrow . When receiving all of the medications, your child will need to be in the hospital.

Days before Transplant	Study plan
21 - 19	Medication: Alemtuzumab
18 - 9	Rest days
8 - 4	Medication: Fludarabine
3	Medication: Melphalan
2 - 1	Rest days
0	Transplant Day

After the transplant, your child will continue to get medications to help the donor cells grow and prevent complications from the transplant. Your child will continue to receive the medications for at least six months after the transplant. Treatment may be necessary in some patients for longer than six months. Study doctors will also do tests to make sure your child’s organs are working well.