

Assent to Participate in Research

Reduced Intensity Conditioning for Haploidentical Bone Marrow Transplantation in Patients with Symptomatic Sickle Cell Disease

Study Title: Reduced Intensity Conditioning for Haploidentical Bone Marrow Transplantation in Patients with Symptomatic Sickle Cell Disease

Protocol: BMT CTN 1507

A. Why am I here?

We are inviting you to join our study because you have severe Sickle Cell Disease (SCD) or because you've had a stroke from the disease. You are now getting blood transfusions, hydroxyurea or other medicines. There is another treatment for sickle cell disease called bone marrow transplant (BMT).

SCD is a blood disease. In SCD, the red blood cells, which are normally shaped like a donut, become sickle shaped. When this happens, they can get stuck in your blood vessels. This can cause pain and damage in different body parts.

BMT uses blood making cells from another person to replace your cells that are not healthy. Donor is the name for a person who gives some of their cells for a transplant. In order for them to give you their cells, their DNA has to match yours.

B. Why are you doing this study?

We know that a transplant can cure SCD but we don't know if it works when the donor is not a perfect match. We are doing this study to see if a transplant with a donor who is related to you, but is not a perfect match, is safe and if it makes you better.

C. What will happen to me?

If you say you want to be in the study, we will ask you to:

- Have check-ups with the study doctors
- Give some blood (about 3 teaspoons)

- If you agree, we will collect extra blood (about 4 teaspoons) at 5 different times. Your blood samples will be used for research about transplant in patients with sickle cell disease. Your samples will be sent to a lab for an important study. All research samples will be tied to a number and researchers testing your samples will not be able to identify you.

We will watch you carefully for fevers, any sign of infection or other problems.

The study will be done over 2 years.

Before your transplant, you will get a small bendable tube put in your chest in the operating room (you will be asleep for this). The small tube makes it easier for you to get your medicines. It will also make it easier for drawing blood for tests because you will not be poked.

We will give you medicines that will help make the cells from your donor grow in your body. These medicines might make you feel sick. You might throw up, lose your hair, or get sores in your mouth.

You will get cells from your donor. This is your transplant. Your new cells will come from your donor's bone marrow. The cells will make new and healthy cells in your body. Because your donor is not a perfect match, you will also get medicines after the transplant to stop the donor cells from attacking your body. This is a problem called graft-versus-host disease (GVHD).

GVHD happens when the donor cells attack your body. It can give you diarrhea, a skin rash, make you feel sick and throw up, or make you not feel hungry. Your doctors will give you medicines to try to make sure you don't get GVHD.

You will stay in the hospital for several days before your transplant and for about 4 weeks after your transplant. After you go home, you will need to go back to see your doctor often.

It is possible that your disease will come back. If this happens, your doctor will find another way to treat you.

D. Will it hurt?

For your transplant, we will put a small tube in your chest. It might hurt a little and you might bleed a little. Your doctor and nurses will make sure you feel as little pain as possible. If you get mouth sores or if you get graft-versus-host disease, this can also hurt, but your doctor will give medicine to help with the pain. These problems usually will get better after a while.

When you have your blood taken with a needle, it may feel like a pinch. It will hurt for a minute and sometimes the place where the needle went might be red and sore. You might get a little bruise from the needle but it goes away in a few days.

The medicines you get might also make you sick. You might feel sick to your stomach or throw up. You might feel tired and your body might hurt. But your doctor will give you other medicine to help you feel better. Also, you might lose your hair. But it will grow back after you are done taking the medicines.

E. Will the study help me?

We don't know if the study will help you or not. What we learn from it might help other people in the future.

F. What if I have questions?

You can ask any questions that you have about the study. If you forget to ask a question and think of it later, you can call me [insert office number]. You can also ask your question the next time you see me.

You can call the study office at any time to ask questions about the study.

G. Do I have to be in this study?

If you don't want to be in the study, you need to tell us and your parent or guardian. Your doctor will not be angry or upset if you don't want to join.

Whether you are in the study or not, you will still need to have treatment for SCD. There might be other studies for sickle cell disease you can join, or a different kind of transplant

You can say yes now and change your mind at any time. Your doctor will not be angry if you change your mind.

Please talk this over with your parent or guardian before you decide if you want to be in the study. We will also ask your parents or guardian to give their permission for you to join this study.

Writing your name on this page means that you agree to be in the study, and know what will happen to you. If you decide to quit the study, all you have to do is tell the person in charge.

You and your parent or guardian will get a copy of this form after you sign it.

Signature of Participant

Date

Printed Name of Participant

Signature of Researcher

Date

Printed Name of Researcher