

Assent to Participate in Research (12 to 17 years of age)

[Insert site logo and/or address]

Study Title: Hematopoietic Cell Transplantation Using Treosulfan-Based Conditioning for the Treatment of Bone Marrow Failure Diseases

Protocol: BMT CTN #1904

Principal Investigator: [Insert site PI]

Source of Support: National Heart, Lung, and Blood Institute (NHLBI) of the National Institutes of Health (the NIH), Bethesda, Maryland

A. Why am I here?

You're being asked to join because you have a bone marrow failure disorder that can be treated with a blood or marrow transplant (BMT).

B. Why are you doing this study?

We're doing this study to see if the drug treosulfan with the drugs fludarabine and Rabbit antithymocyte globulin (rATG) given before a BMT will cause fewer complications.

C. What will happen to me?

We'll ask you to do a few things if you join the study.

1. You'll have several tests to see if you can be in the study. These tests are part of your regular care. They would be done even if you decide not to join this study.
2. Next, you'll receive drugs to prepare your body for BMT. These include the drugs treosulfan, fludarabine and rATG.
3. Then, you'll get donated blood-forming cells for your donor through your central venous line (CVL).

4. Your doctor may decide to give you other medications throughout the study to help with any side effects or discomfort.
5. Lastly, you'll have more tests to see how your body is doing. You'll have these tests every few weeks or months for up to 1 year. We'll also ask you how you're feeling and how well you're able to do the things you normally do each day.

D. Will it hurt?

You may have side effects during the study. Side effects can be mild, severe, or even life-threatening. In some cases, side effects can last a long time or may never go away.

Your health care team may give you medicine to help with some side effects, like an upset stomach.

E. Will the study help me?

This study might help you have less severe complications after BMT than the regular type of BMT that does not use the new medicine called treosulfan.

F. What if I have questions?

Your doctors and nurses will answer your questions. It's important that you get all of your questions answered.

If you forget to ask a question but think of it later, you can call your doctor at [insert office number]. You can also ask your question the next time you see your doctor.

You can call the study office at [insert office number].

Be sure to get all of your questions answered.

G. Do I have to join this study?

You do **not** have to join this study. Tell your doctor and your parents or guardians if you don't want to be in the study. Your doctor won't be angry with you.

It's okay if you say yes now and change your mind and say no later.

You'll still need treatment for your sickness if you don't join this study.

Talk to your parents or guardians before you decide if you want to join this study. We'll also ask your parents if it's okay for you to join.

Extra studies: Blood Draws and Surveys

These are extra studies. You can join in 1 or more of these extra studies. You can join the main study and say no to these extra studies. Your doctor won't be angry with you if you don't want to join the extra studies.

1. Treosulfan Pharmacokinetics (PK) Research Study

We will collect blood samples from you before and after you receive treosulfan. A total of 12 blood samples (6 on each day of infusion) will be collected.

- Not Applicable - accrual for patient age group has been met (to be marked only by the person obtaining consent, if appropriate).
- I agree to give blood samples for the treosulfan research study.
- I do **not** agree to give blood samples for the treosulfan research study.

2. Biological Research Study

The goal of this study is to develop better treatments for bone marrow failure and its complications. We will collect extra blood and/or bone marrow at the same time these samples are already being taken. There are no extra needle pokes involved in this study. These cells will be grown in a lab and kept long term. If you provide samples and do not continue to transplant, your samples will still be stored for research. We will study why blood production is impaired.

- I agree to give blood samples or marrow samples (if a bone marrow is being done for clinical purposes) for the biological research studies.
- I do **not** agree to give blood samples or marrow samples (if a bone marrow is being done for clinical purposes) for the biological research studies.

3. Genetic Testing Research Study (As deemed appropriate by physician.)

The purpose of this study is to figure out what genetic mutation is causing your bone marrow failure, if it is not known already. We will collect an extra blood sample at the same time blood is already being taken. There are no extra needle pokes involved in this study.

- Not Applicable - patient not eligible (to be marked only by the person obtaining consent, if appropriate).
- I agree to give blood samples for the genetic testing research study.
- I do **not** agree to give blood samples for the genetic testing research study.

4. Quality of Life Survey Study

You'll take a survey asking you about your life and how well you can do your normal activities.

- I agree to participate in the optional quality of life questionnaires (both the parent and patient questionnaires if applicable).
- I do **not** agree to participate in the optional quality of life questionnaires (both the parent and patient questionnaires if applicable).

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Writing your name on this page means that you agree to be in the study. 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 (MM/DD/YYYY)

Printed Name of Participant

Signature of Researcher

Date (MM/DD/YYYY)

Printed Name of Researcher