

# PROTOCOL SUMMARY



Frequently asked questions about BMT CTN 2207, a study of upfront bone marrow transplant for patients with severe aplastic anemia

Brought to you by the BMT CTN Patient and Caregiver Advocacy Committee

### What is being studied?

Doctors want to find out if bone marrow transplant works as the first treatment (otherwise known as upfront treatment) for people with severe aplastic anemia. The study is for patients who do not have a related donor whose cells fully match theirs.

Why treat severe aplastic anemia with a half-matched related or unrelated donor transplant before other forms of treatment?

Upfront transplant for severe aplastic anemia has previously been limited to patients with a fully matched related donor. Smaller studies have shown that transplant with a half-matched related donor (called haploidentical) or an unrelated donor, who fully or partially matches the patient, can be effective. This study is being done to confirm those results so haploidentical and matched unrelated donor transplant can be used as a first treatment option for severe aplastic anemia therapy.

## What is the goal of the study?

To see how many patients are living after transplant **without**:

- Serious graft-versus-host disease (GVHD). GVHD happens when the donor's cells (the graft) start to see your body (the host) as different and attack your organs and tissues
- A need for another treatment to cure their severe aplastic anemia

# What if I don't want to be in the study?

There are other treatment options you can discuss with your doctor, such as medications and blood transfusions. There may also be the option to get a bone marrow transplant outside of this study.

# What is different if I join the study?

You will provide blood samples for research (optional) and answer Patient-Reported Outcomes surveys. The transplant method could be similar if you receive a transplant outside of this study. However, the study may be the only current option to get an <a href="mailto:upfront">upfront</a> transplant from a haploidentical or unrelated donor. Ask your doctor for all your options.

#### Are there extra tests and doctor visits?

You'll need to have several tests to see if you can be in the study and have a transplant. These tests would be done for a bone marrow transplant, even if you decide not to join this study. There are additional blood samples for research, which are optional.

### Are there extra costs for participating?

Doctor visits for this study are standard care and will be billed to your health insurance company. If there are extra research tests or costs for this study, you or your health insurance company will not be charged.

#### What are the risks?

There are many potential risks of having a transplant, whether you choose to participate in this study or not. You may have side effects during the study. They can range from mild to severe. Read more about the risks in the consent form. Then, tell your doctor about your questions or concerns.

### Are there potential benefits?

A transplant may cure your severe aplastic anemia.

### How long would I be in the study?

For about 1 year after your transplant.

### I think I want to join. What's next?

- 1. First, you will talk to your doctor about the study and review the consent form.
- 2. If you agree to join, you will sign the consent form.
- You will have screening tests done, which includes making sure that you have a haploidentical or unrelated donor available. You will also complete a pretransplant survey.
- 4. You will get a transplant.

# What if I want to leave the study after I join?

Being in this study is your choice. If at any time you are considering leaving the study, talk to your study doctor about your health and safety.

# What if I have questions? Where can I go for more information?

Your transplant doctor and study team will be able to answer your questions. You can also access these resources:





Study info on BMT CTN website

ClinicalTrials.gov

Clinical Trial IDs: BMT CTN 2207; ClinicalTrials.gov NCT06517641

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