

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

BMT CTN 2207

A Phase II Trial of Non-Myeloablative Conditioning and Transplantation of Haploidentical Related, Partially HLA-Mismatched, or Matched Unrelated Bone Marrow for Newly Diagnosed Patients with Severe Aplastic Anemia

A. Why am I here?

We invite you to join this clinical trial, also known as a research study. You are being asked to join because:

- You have untreated severe aplastic anemia (SAA)
- Your SAA can be treated with an allogeneic transplant. This process uses healthy blood forming cells donated by someone else to replace the unhealthy ones you have.
- You do **not** have a related donor whose cells fully match yours, but you have a donor whose cells half-match yours (this is called haploidentical) or an unrelated donor who fully or partially matches you.

B. Why are we doing this study?

We are doing this study to see how well transplant works for people with untreated severe aplastic anemia. We plan to enroll 60 patients who are between the ages of 3 years old and 75 years old on this study.

Aplastic anemia is a life-threatening condition where the immune system attacks the bone marrow, preventing it from making the normal amount of blood cells. Treatments for SAA try to repair the abnormal immune system attack and let the bone marrow make the normal amount of blood cells. This can be done with a bone marrow transplant or with medications to control the immune system.

C. What will happen to me if I join the study?

If you join, you will:

- Be in the study for 1 year after the transplant.
- Take surveys.
- Before your transplant:
 - Give you medicines to help your body get ready for the transplant. The medicines might make you feel sick. Your doctor will watch your health closely.
- After your transplant:
 - We will also give you medicines to help your body adjust to the new cells. The medicines might make you feel sick. Your doctor will keep watching your health after your transplant.

D. Will it hurt?

- We may need to draw blood from your arm via a needle, so this will hurt for a few seconds. It may be a little red and sore where the needle went in. You may have a little bruise, but it will go away in a few days.
- You could also have side effects from the medicines given to you such as an upset stomach.

E. Answering questions about how you feel

We will ask you some questions about how you feel and how well you are able to do the things you normally do each day if you join the study. Some of the questions or topics may upset you. If this happens, it is important to tell your doctor and care team, because they will not be able to see your answers to these questions on the survey. The survey answers will not be shared with anyone until after the study is completed. If you would like, your doctors can put you in touch with someone who can help if you are feeling very upset. You just have to tell them how you are feeling.

F. Will the study help me?

You may or may not benefit from being in the study. Knowledge gained from this study may help others.

- **Possible Risks:** You may have side effects during the study, and they can vary from mild to severe. Your health care team may give you medicine to help with certain side effects. A common side effect of transplant with donor cells is GVHD (graft-versus-host-disease). Sometimes, the donor's cells (the graft) start to see your body (the host) as different. Therefore, the donor cells may attack your organs and tissues, causing GVHD. It is a medical condition that can be very serious. Your interventions include medicine to help prevent severe GVHD from happening. GVHD may still happen despite prevention, and you may need treatment.

- **Possible Benefits:** If this study works for you, you may need less treatment for your SAA. The information from this study will help doctors learn more about transplants for SAA. It could also help people with SAA who may need a transplant in the future.

G. Key points:

- Being in any research study is your choice.
- You may or may not benefit from being in the study. Knowledge gained from this study may help others.
- If you join the study, you can quit at any time. If you decide to quit the study, it will not affect your care at [name of facility or institution].
- Ask the study staff questions about anything you do not understand, or if you would like more information. You can ask questions now or at any time.
- Every treatment option has benefits and risks. Talk with your doctor about your treatment choices before you decide if you will take part in this study.
- Take time to talk about the study with your doctor, study staff, and your family and friends. It is **your** choice to be in the study. No one can force you to join this study. If you decide to join, please sign the end of this Assent Form. You'll get a copy to keep.

H. What if I have questions?

Ask your doctors and nurses any questions you have; no question is too big or small. 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].

It is your body, so ask questions. Learning more can help you feel comfortable with your treatment.

I. Do I have to join this study?

You don't 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 mad at you.

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

You'll still get care and treatment if you don't join this study.

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

J. What if I do not want to join the study?

If you do not want to join the study, your doctor will talk to you about other treatment options.

K. Optional Blood Samples for Future Research

You can choose to give blood for future research studies if you want to.

If you agree, extra blood will be taken from you and sent to a lab where it will be stored. Your name won't be on the blood we send.

The extra blood will be taken:

- Before your transplant:
 - We will collect (or draw) extra blood 1 time before your transplant. We will draw the blood using your central line if you have one. We will try to draw it when you are already having other blood tests done.
- After your transplant:
 - We will draw extra blood about 5 times, at 1, 2, 3, 6, and 12 months, after your transplant. We will draw the blood using your central line if you have one. We will try to draw it when you are already having other blood tests done.

Minor Assent

Sign your name on the line below if you agree to be in this study. You can also let us know if you want to allow us to take extra blood from you.

Yes, you may take extra blood from me for the future.
 No, you may not take extra blood from me for the future

If you change your mind, 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