

Assent to Participate in Research (7 to 11 years of age)

BMT CTN 2402

Hematopoietic Cell Transplant and Gene Therapy for Non- Malignant Blood Disorders Biobank Resource (The 'HOPE Resource')

Participant's Name: _____ Date: _____

Dr (s). [insert Dr (s) names here] is/are studying serious blood problems.

We are asking for your help because we want to learn more about serious blood disorders like yours . We also want to know why some treatments work for some kids but not others.

If you agree to be in this study, we will ask you to give some of these during your regular doctor or hospital visits:

- Blood, usually from your arm or central line
- A little bit of stool (poop)
- About 10 strands of your hair, and/or about 10 nail clippings and/or a small piece of your skin (just one time)
- A bone marrow sample (only if already being collected)

Your doctor will have regular check-ups with you to see how you are doing after your treatment.

When you are in a study, sometimes good things and bad things can happen. These things may or may not happen to you. Other things may also happen that your doctor does not know about yet.

Here are the bad things that can happen:

- You might feel uncomfortable, afraid, lonely, or hurt. You can stop the study at any time you want to.
- When you give blood, it might hurt a little bit, and you might get a bruise. But, we will only take blood when you're already doing it for your treatment. Your doctor will collect only the amount of blood that is safe for you to give. However, some patients may need a transfusion (additional or earlier than usually scheduled) to replace blood given to your doctors to help you feel better and for this research study.
- You might get an infection from giving nail clippings, hair and skin.

- If you agree to have a small piece of skin taken, you may have pain and soreness to the area. You could also have bruising, bleeding and scarring. There is a small chance of infection from where the skin was taken.

Here are the good things that can happen:

- This study may not help you at this time but can help doctors learn about ways to help other kids that have an illness like yours in the future.

You do not have to be in this study if you do not want to. If you say ‘yes’, and change your mind, you may stop being in this study at any time. Remember, being in this study is up to you.

Here’s what you need to know about your samples:

- Your samples will be kept in a safe place called a repository. Researchers can use them for approved research studies.
- Your samples will be kept until they are used up or until they are no longer needed. Then they will be destroyed.
- After you get all of your stem cells, there might be some left over in the bag. If there are, the bag can be rinsed with a clean liquid. Any extra cells from the rinse can be saved for research later. Usually, these extra cells are thrown away with the bag, so this will not take away any of the cells you need from your transplant. We may also take a small part of the bag that was prepared for your transplant and save it for research. This sample will not have your name on it. It will be used to learn more about transplants, cell therapy, and other studies. The sample will be kept safe in the same place as other samples from this study.
- If you change your mind later and want your samples destroyed, you can contact [insert contact info]. We will try to remove and destroy them, but sometimes we cannot. For example, if your samples have already been used in research, that information cannot be taken back.

Talk with your grown ups before you decide. We will also ask your grown ups if you can be in this study. But even if they say “yes,” **you** can still say no. Your doctor will still take care of you.

You can ask any questions that you have. If you have questions later, your grown ups can call your doctor.

[insert number (s) here]

Optional Samples

Giving other samples (like skin, hair, nails, bone marrow, or stool) is my choice. These samples will also have your name removed and stored for research. You can say “YES” or “NO” for each sample by checking the box.

Bone Marrow

Why: Bone marrow is the soft part inside your bones that makes blood cells. It helps doctors and researchers learn how healthy your blood-making cells are and how your treatment is working. Blood samples only show older cells, but bone marrow shows younger cells that are important to study.

How: If you are already having a bone marrow test as part of your care, we will ask to take a little extra sample for research. We may also use any leftover tissue from your regular test. This will only make the test last a few seconds longer.

- ☐ **YES** – I agree to give a bone marrow sample for research
- ☐ **NO** – I do not agree to give a bone marrow sample for research

Skin, Hair, and Nails

Why: All your cells have something called DNA, which is like instructions that tell your body how to grow and work. Sometimes DNA changes as you grow. By looking at your skin, hair, and nails, researchers can see if these changes happened before you were born or later in life. This helps them understand your disease and your treatment better.

Skin Biopsy

How: We will take a tiny piece of skin about the size of a pencil eraser. First, we clean the area and give you medicine to make it numb so it doesn't hurt. Then we use a small tool to remove the piece of skin. After that, we close the spot with a strip or a stitch and put on a bandage. This takes about 10 minutes.

- ☐ **YES** – I agree to give a skin sample for research
- ☐ **NO** – I do not agree to give a skin sample for research

Hair Plucks (10–15 hairs)

How: We gently pull out a few hairs with the root. You can do this yourself or have a research helper or other grown-up do it. This takes about 5 minutes.

- ☐ **YES** – I agree to give hair samples for research
- ☐ **NO** – I do not agree to give hair samples for research

Nail Clippings (8–10 nails)

How: We clip some nails with regular nail clippers. You can do this yourself or have a research helper or other grown-up do it. This takes about 5 minutes.

- ☐ **YES** – I agree to give nail samples for research
- ☐ **NO** – I do not agree to give nail samples for research

Stool (Poop)

Why: Your poop has bacteria and chemicals that can affect your health. Studying it helps researchers learn how food, medicine, and bacteria in your gut may change your transplant or gene therapy results.

How: You collect a small amount of poop at the hospital, clinic, or home. If at home, we give you a kit and instructions.

- ☐ **YES** – I agree to give a stool sample for research
- ☐ **NO** – I do not agree to give a stool sample for research

Sign and write your name on the line below if you want to be in this study. You can also tell us if you want to give extra blood to help. Remember, you can change your mind at any time. You can keep a copy of this form at home.

Minor Assent

Signature of Participant

Date (MM/DD/YYYY)

Printed Name of Participant

Signature of Researcher

Date (MM/DD/YYYY)

Printed Name of Researcher