

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

BMT CTN 2402

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

Study Title: Hematopoietic Cell Transplant and Gene Therapy for Non-Malignant Blood Disorders Biobank Resource (HOPE Resource)

Protocol: BMT CTN 2402

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

1. Why am I being asked to participate?

You’re being asked to join this biological (biospecimen) and data collection (nontreatment) study because you have a serious blood disorder that is being treated with a blood or marrow transplant (BMT) or gene therapy.

2. What is this study about?

We want to know:

- Why certain treatments for your disease do not work well.
- Understand what causes your serious blood disorder and others and why some people need special treatment.
- Why people respond differently to treatment.
- New ways to treat serious blood disorders.

Approximately 375 bone marrow transplant and gene therapy recipients will be in this study. Study participants will be followed for up to 5 years through March 30, 2031. It is possible the study may be able to continue past March 30, 2031. If that occurs, we will ask you to consent to continue participating past March 30, 2031.

3. What will happen to me?

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

- 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.
- Once you are on the study, someone will ask if the following samples can be collected during regular clinic or hospital visits. You'll have these tests every few weeks or months for the first year that are calendar driven. Then tests will be done every year up to 5 years. You are encouraged to discuss each request with your provider prior to collection.
 - blood
 - stool
 - hair (only at one visit)
 - nail clippings (only at one visit)
 - a very small piece of skin (only at one visit)
 - bone marrow sample (only if already being collected)

4. Will it hurt?

When we take your blood, it will hurt for a few seconds and the place where the needle went in might be a little red and sore. You might get a little bruise, but it goes away in a few days. If you have a central line, we will use the line to draw the blood, so you do not need to have the needle poke done. 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) given your underlying anemia.

There are no major risks to collecting your stool samples. There is a small risk of infection from collection of nails, hair and skin.

If you agree to have a skin biopsy, the doctor will remove a very small piece of skin, usually with a special tool, to help researchers learn more about your health.

Most people do well after a skin biopsy, but there are some risks. These may include:

- Pain or Soreness: The area may hurt or feel sore for a few days.
- Bruising or Bleeding: You might get a small bruise or bleed a little where the skin was removed.
- Infection: There is a small chance of getting an infection at the biopsy site.

- Scarring: A small scar may form where the skin was taken.

5. Will the study help me?

This study will not directly help you, but the information learned from this study may help others.

6. 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.

7. Sample Information

The research team will collect blood samples before and after transplant or gene therapy. At the end of this document, you will have the opportunity to agree to additional sample types (skin, hair, nail clippings, bone marrow, stool) for this research study.

Additional information about your samples is as follows:

- Your samples will not have your name on them or be linked to you in any way. The results of the research, including any genetic studies, will not be shared with you.
- Your samples will be stored in a secure place called a repository, managed by the BMT CTN. Researchers in the scientific community may use them for approved studies.
- If there is any leftover stem cell product after your transplant, the bag may be washed with a sterile solution to collect any remaining cells. These leftover cells are normally thrown away, so this process will not take away any cells you need for your transplant. The leftover cells will be de-identified and stored for future research related to transplant outcomes, cell therapy, and similar studies.
- Your blood and cells contain genetic information called DNA. DNA is like a blueprint that tells your body how to grow and work. It includes traits like hair and eye color and how your body responds to things like infections. DNA can also have changes, called mutations, that happen over time and may affect how genes work. Scientists may study your DNA to learn if certain genes or changes can predict who will have good results or complications after transplant or gene therapy.
- Scientists may use a method called Genome-Wide Association Study (GWAS). GWAS looks at many genes across lots of people to find small changes that might be linked to disease or transplant outcomes. Your samples or your donor's samples may be used in

these studies, but your name will never be linked to the results, and you will not receive any results.

- If your samples are used in these studies, the results will go into a public research database called the NIH Genotype and Phenotype Database, managed by the National Center for Biotechnology Information (NCBI). This database will not include your name or anything that can identify you, although genetic results could theoretically include information about you.
- There is a federal law called the Genetic Information Nondiscrimination Act (GINA). This law makes it illegal for health insurance companies, group health plans, and most employers to use your genetic information to treat you unfairly. However, this law does not cover life insurance, disability insurance, or long-term care insurance.
- Your samples will be stored until they are used up or no longer needed, and then they will be destroyed.
- If you change your mind later and do not want your samples stored or shared, you can contact [insert contact info]. We will do our best to remove and destroy your samples, but sometimes we cannot. For example, if your samples have already been used in research or if we cannot identify them, we will not be able to retrieve them.

8. 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 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.

I understand that drawing additional sample types beyond blood is optional for this study. All identifying information will be removed from the optional samples below and stored for future research use. The following optional sample types, reason for collection, and explanation of how the sample is collected are given below with my decision to agree or disagree to provide the sample by placing a check in the box for my preference "YES" or "NO".

Bone Marrow:

Why: Your bone marrow contains important information about the health of blood-forming cells, how gene therapy or donor immune cells are working, and whether there are small genetic changes that could affect transplant results. This information may not be found in blood alone because blood mostly has older cells, while early cells can only be studied with a bone marrow sample.

How: If you are already having a bone marrow biopsy for your medical care, we ask permission to take a small extra sample for research and to use any leftover tissue from the usual procedure. Since you are already having the biopsy, collecting the extra sample from the same spot will only add a few seconds, usually less than one minute to the procedure.

- ☐ **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 Biopsy/Hair Plucks/Nail Clippings:

Why: Research on rare disorders looks at genes and changes in genes that can be different between people and even between different types of cells in the body. Usually, all the cells in your body have the same genetic information (DNA) when you are born, but as you grow older, some cells can develop changes in their genes. For people with blood disorders, these changes can happen only in blood cells, which makes it hard for researchers to know if the changes were there at birth or happened later. To help answer this question, researchers may collect other cell types, like skin, hair, or nail clippings, and compare their DNA to the DNA in blood cells. If the DNA is the same in all cell types, it means the changes were likely present at birth. If blood cells have a change that other cells do not, it means the change happened after you were born. This information helps researchers better understand your disease and how treatments work.

Skin Biopsy

- **How:** Skin cells will be collected one time before your transplant or gene therapy by doing a small skin biopsy. Whenever possible, this will be done at the same time and place as another procedure you are already having, like a central line placement or bone marrow biopsy. If that is not possible, it can be done as a separate procedure, usually on the upper inner arm. First, the skin will be cleaned to make it sterile, and then a small injection of medicine will numb the area so you do not feel pain. A tool like a tiny cookie cutter will then remove a piece of skin about the size of a pencil eraser. To close the small hole, the doctor will use a steri-strip or a small stitch and then put on a bandage. The whole process 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 total)

- **How:** Hairs will be plucked once pre-transplant or pre-gene therapy to include the root by yourself, a grown-up, or a research coordinator if you prefer. This process 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 Clipping (8 – 10 total)

- **How:** Samples of your nails will be clipped once pre-transplant or pre-gene therapy with standard nail clippers by yourself, a grown-up, or a research coordinator if you prefer. This process 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:

Why: Your stool (poop) contains a material which can impact your overall health and provide information on your health status. For example, the types and amounts of bacteria in your stool may impact the amount of inflammation in your intestines and how you absorb food and medications. Understanding the impact of stool bacterial and chemical changes detected in the stool may give researchers information on how these differences could impact the outcomes of bone marrow transplant or gene therapy.

How: A small amount of stool that you collect at the hospital, clinic, or home would be used for research purposes. If collected at home, you will be given collection materials and instructions.

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

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