

Related Donor Informed Consent to Optional Research Samples

BMT CTN 2402

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

Your Name: _____

Principal Investigator: *Insert local PI information*

Sponsor: This study is sponsored by the National Institutes of Health, through the Blood and Marrow Transplant Clinical Trials Network (BMT CTN)

1. INTRODUCTION

You are being invited to take part in a biorepository nontreatment study because you are a related bone marrow or stem cell donor. If you choose to participate, the study will involve the following one time collections prior to your donation of bone marrow or stem cells:

- **Blood Collection (required):** This includes a Complete Blood Count (CBC) and a sample of peripheral blood.
- **Normal DNA Sample (optional):** We will collect a small sample of your genetic material from another normal tissue using nail clippings (8–10) and/or hair plucks (10–15).

This consent form is for a research study focused on enrolling approximately 100 related donors for individuals with serious blood disorders. The goal of this study is to support future research in rare diseases and to help identify potential new treatments. To achieve this, we will collect samples from both healthy donors and patients undergoing transplant or gene therapy.

It's your choice to participate in the study. Even if you say 'no' to giving samples for this research study, the patient can still receive a transplant from you. If you agree to participate, collection of research samples will be prior to collection of your bone marrow or stem cell (Hematopoietic cell) donation.

- Your name will be removed from the research sample and given a bar code. This bar code helps link your sample to de-identified data and other samples collected on this study by the Data and Coordinating Center for the Blood and Marrow Transplant Clinical Trials Network (BMT CTN DCC). Only the research

team at your hospital and/or your donor center will be able to link you to the research sample.

- The blood samples will be shipped on the day of collection to the BMT CTN Biorepository for processing and initial storage.
- Your research samples will be stored in a repository designated by the BMT CTN and may be used for approved research studies by investigators in the broader scientific community. Samples will be kept until they are used. If you change your mind and no longer wish to have us store or share your data and biospecimens, you should contact [insert contact info]. We will do our best to honor your request and to retrieve any data and biospecimens and destroy them. However, there may be times we cannot. For example, if we do not have a way to identify your data and biospecimens we will not be able to retrieve them. In addition, if the data and biospecimens have already been used for new research, the information from that research may still be used.
- Your name and other information that could directly identify you (such as address or social security number) will not be used. Researchers have a duty to protect your privacy and to keep your information confidential.
- In addition to understanding the types and functions of cells during therapy, your blood and cells have genetic information, called DNA, which contains inherited information, like a blueprint, about the structure of your human body traits such as hair and eye color, but also how your body interacts with environmental exposures such as infections and toxins. DNA also contains changes or mutations that are acquired over a person's lifetime and may impact how the gene functions. DNA from your stored samples may be studied by scientists to learn if some genes or gene changes predict who will have successful outcomes versus serious complications of transplantation or gene therapy.
- Genome-wide association studies (GWAS) are a way for scientists to identify genes involved in human disease. This method searches the genome across a large number of people for small genetic changes that are more common in people with a particular disease than in people without the disease. Each study can look at hundreds of thousands of genetic changes at the same time. Researchers use data from this type of study to find genes that may raise a person's risk of developing a certain disease. You and/or your donor samples may be included in GWAS research, but all genetic information identified will not be linked to your identity nor will any results be given to you through the course of the study.
- If your coded samples are used in such a study, the researcher is required to add your test results and sample information into a shared, public research database. This public database is called the NIH Genotype and Phenotype Database, and it is managed by the National Center for Biotechnology Information (NCBI). The NCBI will never have any information that would identify you, or link you to your information or research samples, although the results of genetic studies could theoretically include identifying information about you.
- A federal law called the Genetic Information Nondiscrimination Act makes it illegal for health insurance companies, group health plans, and employers of 15 or more persons to discriminate against you based on your genetic information. Health insurance companies and group health plans may not request your genetic information that we get from this research. This means that they must not use your genetic information

when making decisions regarding your health insurance. This federal law will not protect you against genetic discrimination by companies that sell life insurance, disability insurance, or long-term care insurance.

- Samples will be stored until they are used up or until it is determined that they are not likely to be used in future research, at which time they will be destroyed.

How can I find out about the results of the research?

You will not have any direct health benefits from providing your specimens for future research. It will probably take a long time for the research performed to be used to produce health-related information that we will know how to interpret accurately. For this reason, and because we will not know who the individual sample donors are, we will not be able to give you individual results from studies that may be conducted using the specimens. Knowledge from future research studies is likely to yield information that is more widely or generally applicable and not specific to an individual.

This Consent Form will tell you about the purpose of the samples for research, the possible risks and benefits, other options available to you, and your rights as a research participant.

Everyone who takes part in research at [insert facility name] should know that:

- Being in any research study is voluntary.
- You will not directly benefit from being in the study. Knowledge we gain from this study may benefit others.
- If you give blood and/or other samples for research, you can change your mind at any time.
- Please ask the study staff questions about anything that you do not understand, or if you would like to have more information.
- You can ask questions now or any time during the study.
- Please take the time you need to talk about the study with your doctor, study staff, and your family and friends. It is your decision to provide samples for research. If you decide to join, please sign and date the end of the Consent Form.

The National Institutes of Health (NIH), through the Blood and Marrow Transplant Clinical Trials Network (BMT CTN), are giving staff support and money to conduct this research study.

The BMT CTN will lead the research study and, along with the NIH, will make decisions about how to manage the study.

2. STUDY PURPOSE

The purpose of this study is to support future research in serious blood disorders and to help discover new treatments.

3. RIGHT TO ASK QUESTIONS AND/OR WITHDRAW

You have the right to ask questions about the study at any time. If you have questions about your rights as a study participant or you want to leave the study, please contact:

[insert contact info]

Giving blood and/or other samples for research is voluntary. You can choose not to give samples at any time.

If you choose not to take part or change your mind, it will not affect your donation process or the treatment of the patient in any way.

Samples and information that have already been used for research cannot be taken back or destroyed.

Your study doctor and study staff will be available to answer any questions that you may have.

4. STUDY TREATMENTS AND TESTS

If you agree to give blood and/or other samples, here is what will happen:

- We will collect an extra blood sample prior to collection of your bone marrow or stem cell donation. The amount of blood collected from you will be 35 mL (about 7 teaspoons).
- We will also collect hair follicles (e.g., you or a researcher will pluck a few scalp or eyebrow hairs) and/or nail clippings (e.g. you or a researcher will clip your nails using a nail clipper) prior to collection of your bone marrow or stem cell donation. The amount of hair follicles (plucks) will be 10-15 and nail clippings 8-10.
- The sample(s) will be sent to the BMT CTN Biorepository for storage. The sample(s) will be given a unique bar code that cannot be linked to you by the researchers testing your samples.

5. RISKS AND DISCOMFORTS

There are no major risks to having your blood drawn, hair and/or nails collected. It can be uncomfortable to have your blood taken and it can sometimes leave a bruise. You might faint, but this is unlikely to happen. Only trained people will take your samples.

Risks of Genetic Testing

In the course of these studies, we may find new genes that are inherited and predict the development of specific illnesses. Once we have obtained your DNA (or genes), we will put the DNA in tubes. These tubes will be labeled with a code and will have no markings to link the tube with you specifically. If we learn anything of importance to our research from this testing, we may publish the results in a medical journal. However, you will not be identified in the article as the patient who provided the blood sample for our testing.

In rare instances, it is possible that we could find out information about a specific gene that could affect you or other members of your family in terms of insurability, employability, or paternity. We

will do everything possible to ensure that your identity and confidentiality will not be breached. As previously mentioned, the code linking your identifying information to the sample will be kept secure by the BMT CTN DCC staff in a password-protected file in a secure location.

6. POSSIBLE BENEFITS

There is no direct benefit to you to join the study, but the knowledge gained from this study may help others.

7. PRIVACY, CONFIDENTIALITY AND USE OF INFORMATION

Your privacy is very important to us. The study doctors will make every effort to protect it. The study doctors have a privacy permit to help protect your records if there is a court case. However, some of your medical information may be given out if required by law. If this should happen, the study doctors will do their best to make sure that any information that goes out to others will not identify who you are.

[Name of Transplant Center] and the organizations listed below will not disclose your participation by any means of communication to any person or organization, except by your written request, or permission, or unless required by federal, state or local laws, or regulatory agencies.

- The National Institutes of Health (NIH), which include the National Heart, Lung, and Blood Institute (NHLBI) and the National Cancer Institute (NCI).
- U.S. government agencies that are responsible for overseeing research such as The Food and Drug Administration (FDA) and the Office of Human Research Protection (OHRP).
- U.S. government agencies that are responsible for overseeing public health concerns such as the Centers for Disease Control (CDC) and federal, state, and local health departments.
- The Data and Safety Monitoring Board (DSMB), not part of [Institution].
- NMDP Institutional Review Board (IRB) is responsible for this study.
- Blood and Marrow Transplant Clinical Trials Network Data and Coordinating Center (BMT CTN DCC), including:
 - The Center for International Blood and Marrow Transplant Research (CIBMTR).
 - NMDP.
 - Emmes, who is coordinating the studies of the BMT CTN.
- Study investigators.

Individuals authorized by the organizations above will have access to your research and medical information. They may use this information for inspections or audits to study the outcomes of your donation. In agreeing to participate, you consent to these inspections. You also consent to allow authorized individuals to copy parts of your records, if required by these organizations.

We may give out your personal information if required by law. If information from this study is published or presented at scientific meetings, your name and other personal information will not be used.

Private information and samples taken during the study will be used for future research. However, samples will not be attached to you or your name in any way and results of the research done with it will not be given to you.

A description of this clinical trial will be available on <http://www.ClinicalTrials.gov>, as required by U.S. Law. This website will not include information that can identify you. At most, the website will include a summary of the results. You can search this website at any time.

For questions about access to your medical records, please contact [name] at [number].

8. ENDING YOUR PARTICIPATION

The study doctor or the study sponsor may stop the study at any time, and we may ask you to leave the study. We may ask you to leave the study if you do not follow directions or if you suffer from side effects of the blood draw or hair plucks/nail clippings. The study sponsor may decide to end the study at any time. If we ask you to leave the study, the reasons will be discussed with you.

Possible reasons to end your participation in this study include:

- You do not meet the study requirements.
- You become unable to donate bone marrow or stem cells (Hematopoietic cells).
- The recipient no longer is eligible to receive bone marrow or stem cells.
- The study is stopped for any reason.

9. PHYSICAL INJURY AS A RESULT OF PARTICIPATION

It's important that you tell your doctor, [investigator's name(s)], or study staff if you feel that you have been injured because you provided blood samples, hair plucks, or nail clippings for research. You can tell the doctor in person or call him/her at [telephone number].

You will get all available medical treatment if you are injured as a result of providing blood samples for research.

You, your health plan, or your family member's (if donating for a family member) health plan will be charged for this treatment for injury. The study will not pay for medical treatment.

In case of injury resulting from providing the blood sample for this study, you do not lose any of your legal rights to seek payment by signing this form.

10. PAYMENT AND STUDY COSTS

You will not be paid for your participation in the research study or for providing samples for research.

You will not be compensated or reimbursed for any extra costs (for example, travel and meals) from taking part in this study.

The visit at which research samples will be collected is standard for bone marrow or stem cell donors and will be covered by the recipient's insurance.

You will not be charged for the collection of the optional sample or for the research tests done with these samples. The costs of shipping your samples will be paid by the BMT CTN.

For questions about your costs, financial responsibilities, and/or medical insurance coverage for your transplant and this study, please contact [Center / Financial Counselor at Number].

11. FOR MORE STUDY INFORMATION

If you need more information about providing samples for research, or if you have problems while you are participating in this study, you can contact the study doctor or his/her staff. They can be reached at the telephone numbers listed here:

[Insert name and contact details].

12. CONTACT SOMEONE ABOUT YOUR RIGHTS

If you wish to speak to someone not directly involved in the study, if you have any complaints about the study, or would like more information about your rights as a research participant, you may contact:

[Insert appropriate contact details].

The ethical aspects of this research study have been reviewed and approved by [name of IRB].

For more information about your rights when providing samples for research, call NMDP Institutional Review Board (a group of people who review the research to protect your rights) at [telephone number].

Statement of Consent for the Study

TITLE: BMT CTN 2402: Hematopoietic Cell Transplant and Gene Therapy for Non-Malignant Blood Disorders Biobank Resource

- I have read and understood this Consent Form. The purpose and description of the research study has been explained to me.
- I have had the chance to ask questions and understand the answers I have been given. I understand that I may ask questions at any time during the study.
- I freely agree to be a participant in the study.
- I have had the chance to discuss my participation in this research study with a family member or friend if I choose.
- I understand that...
 - I may not directly benefit from taking part in the study.
 - My name and personal information will not be identified even if information gained during the study is published.
 - I can leave this study at any time and doing so will not affect my current care or prevent me from receiving future treatment.
 - I will be given a copy of this signed consent form.
 - I do not give up any legal rights by signing this form.
 - Information about me and my medical course will be collected for research. This research data will have all identifying information removed so researchers will not know who I am in this study.
 - A blood sample and possibly hair plucks and nail clippings will be drawn one time for this study time, identifying information will be removed from the samples, and samples

will be stored for future research use. All attempts will be made to collect the blood sample during a blood draw done for your usual clinical needs.

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 checking the box associated with my preference “YES” or “NO”.

Hair Plucks/Nail Clippings:

Why: Research in rare disorders often focuses on understanding genes and how changes in those genes can differ between individuals and even between different cell types in the body. While most cells share the same genetic information (DNA) from birth, some cells may acquire changes over time. For patients with blood disorders, these changes can occur only in blood cells, making it difficult for researchers to determine whether the changes were present at birth or developed later.

To answer this question, researchers collect germline samples that represent your original genetic makeup such as hair or nail clippings. These samples allow comparison between the DNA in blood cells and DNA in other cell types. If the DNA is consistent across all samples, it suggests that any genetic mutations were present at birth. If blood cells show a mutation that is absent in other cell types, it indicates the change occurred after birth in the blood cells only. This information is critical for understanding the nature of the disease and improving treatment strategies.

For related donors, germline samples are equally important. They help confirm whether genetic changes are inherited or acquired, which can guide decisions about donor suitability and provide valuable insights into familial risk factors.

Hair Plucks (10 – 15 total)

- **How:** Hairs will be plucked once pre-transplant or pre-gene therapy to include the root by yourself 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 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

Sign your name on the line below if you agree to be in this study. If you are a legally authorized representative (LAR), sign on behalf of the participant.

Participant Name (or Parent/Guardian/LAR)

Participant Signature (or Parent/Guardian/LAR)

Date (MM/DD/YYYY)

I certify that I have provided a verbal explanation of the details of the research study, including the procedures and risks. I believe the participant has understood the information provided.

Name of Counseling Physician/Staff

Signature of Counseling Physician/Staff

Date (MM/DD/YYYY)

Name of Interpreter

Signature of Interpreter

Date (MM/DD/YYYY)