

PRINCIPAL INVESTIGATOR: Dimana Dimitrova, M.D.

STUDY TITLE: Phase II Trial of Allogeneic Hematopoietic Cell Transplantation for Disorders of T cell Proliferation and/or Dysregulation

STUDY SITE: NIH Clinical Center

Cohort: Donor

Consent Version: 06/04/2024

WHO DO YOU CONTACT ABOUT THIS STUDY?

Dimana Dimitrova, M.D. by phone at 240-858-3647 or email: dimana.dimitrova@nih.gov

This consent form describes a research study and is designed to help you decide if you would like to be a part of the research study.

The remaining document will now describe the research study in more detail. This information should be considered before you make your choice. Members of the study team will talk with you about the information in this document. Some people have personal, religious, or ethical beliefs that may limit the kinds of medical or research interventions in which they would want to participate. Take the time needed to ask any questions and discuss this study with NIH staff, and with your family, friends, and personal health care providers. Taking part in research at the NIH is your choice.

If the individual being enrolled is a minor then the term “you” refers to “you and/or your child” throughout the remainder of this document.

IT IS YOUR CHOICE TO TAKE PART IN THE STUDY

You may choose not to take part in this study for any reason. If you join this study, you may change your mind and stop participating in the study at any time and for any reason. In either case, you will not lose any benefits to which you are otherwise entitled. However, to be seen at the NIH, you must be taking part in a study or are being considered for a study. If you do choose to leave the study, please inform your study team to ensure a safe withdrawal from the research.

WHY IS THIS STUDY BEING DONE?

This study is being done to evaluate the role of allogeneic blood or bone marrow transplant in treating patients with problems with the function of the T cells of their immune system. The study aims to determine if allogeneic bone marrow transplant is effective in patients with these problems while also minimizing side effects and complications.

WHY ARE YOU BEING ASKED TO TAKE PART IN THIS STUDY?

Your relative has a problem with his or her T cells and is eligible to undergo transplantation to try to correct or even cure the disease. Allogeneic hematopoietic cell (blood stem cell and immune cell) transplantation involves the transfer of cells from a person with a healthy immune system to

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the patient. You are being asked to take part in this study because you are eligible to serve as a donor of blood cells for your relative's transplant. If your blood cells are accepted by your relative's body, your cells will help his/her body being to grow normal blood cells and immune cells.

HOW MANY PEOPLE WILL TAKE PART IN THIS STUDY?

Up to 177 people (117 recipients/60 donors) will take part in this research study.

DESCRIPTION OF RESEARCH STUDY

What will happen if you take part in this research study?

Before you begin the study

In order to determine if you are able to be a donor, the following tests and procedures will be performed. If you have had some of these tests or procedures recently, they may or may not have to be repeated:

- Review your medical history and list of current medications.
- Physical exam to include height, weight, blood pressure, heart rate.
- Blood Draws (3 tablespoons): Blood will be drawn from either an arm vein or a central venous access device if you have one. Routine blood tests to check your organ function, blood counts, immune system function, past exposures to infections, and screen for active infections and other routine tests that determine whether you meet the requirements for participating in a specific protocol.
- Pregnancy Test: For individuals who could have children, a pregnancy test will be done (blood or urine sample). You will not be able to participate if you are pregnant.
- Electrocardiogram: An electrocardiogram (ECG) is a test that is performed while you lie still for about 5 minutes. It involves placing electrodes (small stickers that are attached to wires that go to the machine) on the chest and arms/legs and recording the electrical activity of your heart. If you have a lot of hair on your chest, it may hurt a little bit when they remove these stickers.

If you are chosen to be the donor, you will be asked if you are additionally willing to participate in this research study and contribute blood and marrow/peripheral blood cell specimens for research. You may donate blood cells to your relative without participating in this research study.

Research studies will be done on your blood and your donated marrow or peripheral blood cells to evaluate the composition of your immune system and how well it functions. Research studies will also be done to evaluate if you have previously been infected with certain viruses that, once infected, remain in your body for your lifetime. Research studies will also be performed to study how well your immune system controls these viruses. You will be asked to give extra blood (25 teaspoons or ½ cup) and a small amount of your donated marrow or peripheral blood cells (6 teaspoons, taken from the collection bag at the time of donation, after it is determined that the target number of cells to collect has been reached for your relative) for these studies. We will not take a research specimen from your marrow or peripheral blood cell donation if the target number

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of cells to collect is not reached. Although it is safe for adults to give 25 teaspoons of blood at one time, blood sample collection can be spaced over up to three separate blood draws if you prefer to have smaller amounts of blood drawn each time, or if you are a small-framed adult. For pediatric donors, up to a collective total of 2 teaspoons will be collected. The research marrow samples will be collected from you at the time of bone marrow harvest, if that is how you donate the cells, while under anesthesia.

RISKS OR DISCOMFORTS OF PARTICIPATION

The risks and discomforts of participating as a donor include:

Blood draws: Side effects of blood being drawn include pain and bruising in the area where the needle was put in, lightheadedness, or fainting (rarely). When too much blood is taken, one's red blood cell count may drop, causing anemia. We will monitor your blood cell counts. If we find that you have anemia, we will give you treatment for this condition in the form of iron tablets, or a blood transfusion if your red blood cell counts are very low.

Electrocardiogram (ECG): An ECG is a tracing of your heart rate and rhythm. This test is very safe and is performed using wires that are briefly placed on the skin of your chest.

POTENTIAL BENEFITS OF PARTICIPATION

Your participation may help advance our understanding of hematopoietic cell transplantation for T-cell-related problems in general.

STOPPING PARTICIPATION

Your doctor may decide to stop your participation for the following reasons:

- if he/she believes that it is in your best interest
- if the study doctor decides to end the study
- if you lose the capacity to provide consent
- if it is decided to use a different donor for your relative
- If the recipient is removed from the study

You can stop taking part in the study at any time. However, if you decide to stop taking part in the study, we would like you to talk to the study doctor first.

If you decide at any time to withdraw your consent to participate in the trial, we will not collect any additional medical information about you. However, any samples and data generated from the samples that have already been distributed to other researchers or placed in the research databases **cannot** be recalled and destroyed.

WHAT OTHER CHOICES DO I HAVE IF I DO NOT TAKE PART IN THIS STUDY?

You can choose not to donate specimens for research.

Conflict of Interest

The National Institutes of Health (NIH) reviews NIH staff researchers at least yearly for conflicts of interest. This process is detailed in a Protocol Review Guide. You may ask your research team for a copy of the Protocol Review Guide or for more information. Members of the research team who do not work for NIH are expected to follow these guidelines but they do not need to report their personal finances to the NIH.

Members of the research team working on this study may have up to \$15,000 of stock in the companies that make products used in this study. This is allowed under federal rules and is not a conflict of interest.

USE OF SPECIMENS AND DATA FOR FUTURE RESEARCH

To advance science, it is helpful for researchers to share information they get from studying human samples. They do this by putting it into one or more scientific databases, where it is stored along with information from other studies. A researcher who wants to study the information must apply to the database and be approved. Researchers use specimens and data stored in scientific databases to advance science and learn about health and disease.

We plan to keep some of your specimens and data that we collect and use them for future research and share them with other researchers. We will not contact you to ask about each of these future uses. These specimens and data will be stripped of identifiers such as name, address or account number, so that it may be used for future research on any topic and shared broadly for research purposes. Your specimens and data will be used for research purposes only and will not benefit you. It is also possible that the stored specimens and data may never be used. Results of research done on your specimens and data will not be available to you or your doctor. It might help people who have cancer and other diseases in the future.

If you do not want your stored specimens and data used for future research, please contact us in writing and let us know that you do not want us to use your specimens and/or data. Then any specimens that have not already been used or shared will be destroyed and your data will not be used for future research. However, it may not be possible to withdraw or delete materials or data once they have been shared with other researchers.

Protections against misuse of genetic information

This study involves genetic testing on samples. Some genetic information can help predict future health problems of you and your family and this information might be of interest to your employers or insurers. The Genetic Information Nondiscrimination Act (GINA) is a federal law that prohibits plans and health insurers from requesting genetic information or using genetic information. It also prohibits employment discrimination based on your health information. However, GINA does not address discrimination by companies that sell life insurance, disability insurance, or long-term care insurance. GINA also does not protect you against discrimination based on an already-diagnosed condition or disease that has a genetic component.

COMPENSATION, REIMBURSEMENT, AND PAYMENT**Will you receive compensation for participation in the study?**

You will not receive compensation for participation in this study.

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Will you receive reimbursement or direct payment by NIH as part of your participation?

On this study, the NCI will cover the cost for some of your expenses. Some of these costs may be paid directly by the NIH and some may be reimbursed after you have paid. The amount and form of these payments are determined by the NCI Travel and Lodging Reimbursement Policy. You will be given a summary of the policy which provides more information.

Will taking part in this research study cost you anything?

NIH does not bill health insurance companies or participants for any research or related clinical care that you receive at the NIH Clinical Center.

- If some tests and procedures are performed outside the NIH Clinical Center, you may have to pay for these costs.

CLINICAL TRIAL REGISTRATION AND RESULTS REPORTING

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

CONFIDENTIALITY PROTECTIONS PROVIDED IN THIS STUDY**Will your medical information be kept private?**

We will do our best to make sure that the personal information in your medical record will be kept private. However, we cannot guarantee total privacy. Organizations that may look at and/or copy your medical records for research, quality assurance, and data analysis include:

- The NIH and other government agencies, like the Food and Drug Administration (FDA), which are involved in keeping research safe for people.
- National Institutes of Health Intramural Institutional Review Board

The researchers conducting this study and the NIH follow applicable laws and policies to keep your identifying information private to the extent possible. However, there is always a chance that, despite our best efforts, your identity and/or information about your participation in this research may be inadvertently released or improperly accessed by unauthorized persons.

In most cases, the NIH will not release any identifiable information collected about you without your written permission. However, your information may be shared as described in the section of this document on sharing of specimens and data, and as further outlined in the following sections.

Further, the information collected for this study is protected by NIH under a Certificate of Confidentiality and the Privacy Act.

Certificate of Confidentiality

To help us protect your privacy, the NIH Intramural Program has received a Certificate of Confidentiality (Certificate). With this certificate, researchers may not release or use data or information about you except in certain circumstances.



NIH researchers must not share information that may identify you in any federal, state, or local civil, criminal, administrative, legislative, or other proceedings, for example, if requested by a court.

The Certificate does not protect your information when it:

1. is disclosed to people connected with the research, for example, information may be used for auditing or program evaluation internally by the NIH; or
2. is required to be disclosed by Federal, State, or local laws, for example, when information must be disclosed to meet the legal requirements of the federal Food and Drug Administration (FDA);
3. is for other research;
4. is disclosed with your consent.

The Certificate does not prevent you from voluntarily releasing information about yourself or your involvement in this research.

The Certificate will not be used to prevent disclosure to state or local authorities of harm to self or others including, for example, child abuse and neglect, and by signing below you consent to those disclosures. Other permissions for release may be made by signing NIH forms, such as the Notice and Acknowledgement of Information Practices consent.

Privacy Act

The Federal Privacy Act generally protects the confidentiality of your NIH medical records we collect under the authority of the Public Health Service Act. In some cases, the Privacy Act protections differ from the Certificate of Confidentiality. For example, sometimes the Privacy Act allows release of information from your medical record without your permission, for example, if it is requested by Congress. Information may also be released for certain research purposes with due consideration and protection, to those engaged by the agency for research purposes, to certain federal and state agencies, for HIV partner notification, for infectious disease or abuse or neglect reporting, to tumor registries, for quality assessment and medical audits, or when the NIH is involved in a lawsuit. However, NIH will only release information from your medical record if it is permitted by both the Certificate of Confidentiality and the Privacy Act.

RESEARCH-RELATED INJURIES

The NIH Clinical Center will provide short-term medical care for any injury resulting from your participation in research here. In general, no long-term medical care or financial compensation for research-related injuries will be provided by the NIH, the NIH Clinical Center, or the Federal Government. However, you have the right to pursue legal remedy if you believe that your injury justifies such action.

PROBLEMS OR QUESTIONS

If you have any problems or questions about this study, or about your rights as a research participant, or about any research-related injury, contact the Principal Investigator, Dimana Dimitrova, M.D., by phone at 240-858-3647 or email: dimana.dimitrova@nih.gov. You may also call the NIH Clinical Center Patient Representative at 301-496-2626, or the NIH Office of IRB Operations at 301-402-3713, if you have a research-related complaint or concern.



CONSENT DOCUMENT

Please keep a copy of this document in case you want to read it again.

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Adult Research Participant: I have read the explanation about this study and have been given the opportunity to discuss it and to ask questions. I consent to participate in this study.

Signature of Research Participant

Print Name of Research Participant

Date

Parent/Guardian of a Minor Participant: I have read the explanation about this study and have been given the opportunity to discuss it and to ask questions. I give permission for my child to take part in this study.

Signature of Parent/Guardian

Print Name of Parent/Guardian

Date

Signature of Parent/Guardian (*as applicable*)

Print Name of Parent/Guardian

Date

Assent: I have had this study explained to me in a way that I understand, I have been given the opportunity to discuss it, and I have had the chance to ask questions. I agree to take part in this study.

Assent of Minor:

Signature of Minor

Print Name of Minor

Date

Investigator:

Signature of Investigator

Print Name of Investigator

Date

Witness should sign below if either:

1. A short form consent process has been used to enroll a non-English speaking subject or
2. An oral presentation of the full consent has been used to enroll a blind or illiterate subject

Signature of Witness

Print Name of Witness

Date

NIH ADMINISTRATIVE SECTION TO BE COMPLETED REGARDING THE USE OF AN INTERPRETER:

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_____ An interpreter, or other individual, who speaks English and the participant's preferred language facilitated the administration of informed consent and served as a witness. The investigator obtaining consent may not also serve as the witness.

_____ An interpreter, or other individual, who speaks English and the participant's preferred language facilitated the administration of informed consent but did not serve as a witness. The name or ID code of the person providing interpretive support is: _____.