University of Washington

Consent Form

Family Member Subject
Severe Chronic Neutropenia Tissue Bank

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In this document, the term "you" refers to you or your child.

Investigator's Statement

We are asking you/your child to be in a research study. The purpose of this consent form is to give you the information you will need to help you decide whether or not to be in the study. Please read the form carefully. You may ask questions about the purpose of the research, what we would ask you to do, the possible risks and benefits, your rights as a volunteer, and anything else about the research or this form that is not clear. When all your questions have been answered, you can decide if you want to be in the study or not. This process is called 'informed consent.'

Purpose

Neutrophils are white blood cells which prevent and fight infections. The neutrophil count is a measure of the number of these cells in the blood. Neutropenia is a reduction in the number of circulating neutrophils. As the number decreases, the number and frequency of infections increases. When the neutrophil level is very low (that is about one tenth of the normal level or less) for weeks or months, the condition is called "severe chronic neutropenia." Severe chronic neutropenia has many causes and some, but not all, are inherited/genetic diseases. Diseases of the immune system can also cause neutropenia. The exact way in which this condition occurs is not yet known.

The objective of this study is to learn more about the causes of severe chronic neutropenia through the study of blood, bone marrow, and other cells of people with this condition, and their immediate relatives. The study compares the blood samples of people with severe chronic neutropenia in contrast to the samples of their immediate relatives or normal volunteers; it also compares the bone marrow samples of people with severe chronic neutropenia to those of normal volunteers. The study is looking at the genetic causes for severe chronic neutropenia and comparing the results of the subjects with neutropenia to the subjects without neutropenia. There is no direct benefit from these tests but the information learned through this research may be of long-term value in understanding these conditions and developing new treatments.

Procedures

To be included in this study, you must have an immediate relative with the diagnosis of severe chronic neutropenia. If you agree to participate, and after you have signed this Consent Form, your doctor, with your help, will be asked to complete a brief form about your current health to be sent to the University of Washington with your blood or tissue sample. This form asks for some basic information about you, such as your name, address, telephone number, age, sex, and date of birth. In addition, we will also ask for information about your blood counts, your general health, particularly related to your susceptibility to infections, if you are administering Filgrastim, (also called G-CSF, granulocyte-colony stimulating factor or Neupogen) and if other family members have neutropenia. We may need access to your medical records to clarify the medical information sent to the Study.

Your cells will become part of the University of Washington Severe Chronic Neutropenia Tissue Bank for the study of Severe Chronic Neutropenia. The cells and materials from them (DNA, RNA and proteins) will be frozen and kept indefinitely for research including genetic testing. The samples will not be identified with your name, but will have a code number which can only be linked to your personal health data (the information on the form sent with the sample) by one of the researchers listed on the consent form.

Risk, Stress or Discomfort

Genetic Tests

It is theoretically possible that participation in this genetics study might hurt your access to health insurance if information about your involvement and/or results of the study become part of your medical record. Therefore, we will keep all study data out of your medical record by keeping this information completely separate. The decision to learn the results of the study is highly personal and it may be emotionally stressful.

Blood Draws

The blood draw can cause dizziness, weakness, fainting, pain, bleeding, or infection.

Buccal Smear (if applicable)

The Buccal smear is usually painless, with removal of a few cells from the surface of your cheek inside your mouth. A small plastic spatula or spoon is used to gently scrape cells from the inside of your mouth.

Mouthwash Rinse Sample

A mouthwash rinse sample is usually painless. If you have a mouth ulcer or open sore in your mouth the mouthwash may cause a little stinging while you are swishing. The stinging will stop after you spit out the mouthwash.

Subjects older than 18 years old

Skin Biopsy (if applicable)

The skin biopsy is done with a local anesthesia (usually lidocaine 1% up to 1 ml or 1/5 of a teaspoon) injected under the skin to prevent pain. A very small hole, about the size of a pencil lead (2 mm) is created. The spot is covered by a band-aid; it generally heals quickly and only rarely leaves any scar. Bleeding or local infection is possible at the site of the skin biopsy, but rarely occurs.

The risk of the local anesthetic for the skin biopsy is primarily an allergic reaction to the anesthetic agent with redness and itching at the injection site or rarely, on other areas of the body. In the event that any complication occurs, appropriate medical treatment will be provided as indicated.

Alternatives To Taking Part In This Study

You can choose not to participate and not have samples taken for research purposes.

Benefits

You will not directly benefit if you take part in this research study. What we learn from the research may benefit neutropenic patients and society in the future.

Source of Funding

The study team and/or the University of Washington is receiving study drug from Amgen, Inc., and financial support from Emendo Biotherapeutics and NIH grant # 1 R01HL151629-01.

Other Information

Being in this study is voluntary. You are free to refuse to participate and may withdraw at any time without penalty or loss of benefits to which you are otherwise entitled. The samples of your cells will be studied at the University of Washington and in other research institutions, only for the purpose of increasing understanding and improving the treatment of severe chronic neutropenia. These studies are done for research purposes and are not diagnostic tests. All information collected for the study will be strictly confidential. Records with identifying information will have this information removed and given a study number. These files will be kept in a locked filing cabinet. The code that links your name to the study number will be kept in a computer file that will need a password to enter and will be kept indefinitely. The investigators in this study will have access to the password and study files.

Although we will make every effort to keep your information confidential, no system for protecting your confidentiality can be completely secure. It is possible that unauthorized persons might discover that you are in this study, or might obtain information about you. University and government offices sometimes review studies such as this one to make sure they are being done safely and legally. If a review of this study takes place, your records may be examined. The reviewers will protect your privacy. The study records will not be used to put you at legal risk of harm.

We have a Certificate of Confidentiality from the federal National Institutes of Health. This helps

us protect your privacy. The Certificate means that we do not have to give out identifying information about you even if we are asked to by a court of law. We will use the Certificate to resist any demands for identifying information.

We can't use the Certificate to withhold your research information if you give your written consent to give it to an insurer, employer, or other person. Also, you or a member of your family can share information about yourself or your part in this research if you wish.

There are some limits to this protection. We will voluntarily provide the information to:

- a member of the federal government who needs it in order to audit or evaluate the research;
- individuals at the University of Washington, the funding agency, and other groups involved in the research, if they need the information to make sure the research is being done correctly;
- the federal Food and Drug Administration (FDA), if required by the FDA;
- State or local authorities, if we learn of child abuse, elder abuse, or the intent to harm yourself or others.

The Certificate expires when the NIH funding for this study ends. Currently this is 03/31/2024. Any data collected after expiration is not protected as described above. Data collected prior to expiration will continue to be protected.

The results of this research may be published in scientific journals, although no identifying information will be contained in these articles. You will not be paid for your participation in this study.

The sample of your cells will be kept and used for continuing research on neutropenia for an indefinite period. Occasionally, perhaps many years after the sample is obtained, a disease-causing gene or other new information about neutropenia may be found from using your cells.

You have a right to know the results of research with your cells. These results are research tests only and are not diagnostic tests. If you request, we will provide you, your physician, or other health care provider with the results from studies done with your cells. **The results usually take several months, but it can take up to a year to get results.** Having such information given to you is voluntary and you do not have to be told about the results if you do not want this information. The decision to have such information revealed is a highly personal, and it may be stressful. At present we see no specific risk to your employment or insurance status from this research, but we cannot guarantee that there is not a risk. Understanding the results of genetic test is a complex process that may require professional genetic counseling. You can request genetic counseling to understand these results. We will assist you in arranging counseling through your physician or health care provider. If you need further information, you can call us in Seattle at (206) 543-9749 (Audrey Anna Bolyard) or 206 543-7215 (Dr. David Dale). We cannot pay for such counseling.

Dr. David C. Dale, the principal investigator of this study, has conducted research and is a consultant and speaker for Amgen, the pharmaceutical company that produces Neupogen and is an original sponsor of the tissue bank. Dr. Dale also has a financial relationship with EmendoBio, Inc., a biopharmaceutical company that helps support the work of the Tissue Bank described in this consent form. Dr. Dale is Principal Investigator for this study. These financial conflicts of interest have been reviewed by the University of Washington Office of Research.

This research may lead to new drugs, tests or treatments. It may also be used to develop new commercial products. There are no plans to share the profits, if there are any, with you.

We will store your samples to use in future research. We will send our request to do further research with your sample to an Institutional Review Board for approval. This Board protects the rights and welfare of research subjects like you. The Board will determine if we need to contact you and ask your consent to do the research.

At your request, the samples and information about you may be removed from the Tissue Bank and destroyed. You will receive a copy of this consent form.

Compensation for Injury

If you think you have an injury or illness related to this study, contact the study staff, Audrey Anna Bolyard (206-543-9749; long distance 1-800-726-4463) or Dr. David C. Dale (206-543-7215). The researchers will treat you or refer you for treatment. The University of Washington will pay up to $10,000 to reimburse for treatment of physical injury or illness resulting from the study. No money has been set aside to pay for things like lost wages, lost time, or pain. However, you do not waive any rights by signing this consent form. We will bill your insurer for treatment of problems that result directly from your neutropenia or from standard clinical care.

_____ _____
Signature of person obtaining consent Date

_____
Printed name of person obtaining consent

Subject's Statement:

This study has been explained to me. I volunteer to take part in this research. I give permission to the researchers to use my medical records as described in this consent form. I have had a chance to ask questions. If I have questions later on about the research I can ask one of the investigators listed above. If I have questions about my rights as a research subject, I can call the Human Subjects Division at (206) 543-0098. I will receive a copy of this consent form.

If you agree, as indicated by your initials below, a cell or tissue sample will be collected by your physician or health care provider and will be sent to the University of Washington for the Severe Chronic Neutropenia Tissue Bank.

Subjects younger than 18 years old

___ a. A blood sample, up to 30 ml (approximately 2 tablespoons) will be drawn; in children less than 10 years old the maximum sample will be 2 ml/kg (approximately 1 teaspoon per 5 pounds of body weight). The blood sample for research will be drawn at the time of a routine blood draw.

___ b. A buccal smear (a light scraping of cells from your mouth with a small plastic spatula or spoon)

___ c. A mouthwash rinse sample – For Subject 7-18 years old: We ask that you rinse your mouth with water to remove any food particles and then do not drink or eat for one hour. At the end of the hour please swish approximately three teaspoons of mouthwash (mint flavored) or sterile water from side to side in your mouth, continue to swish for about 30-45 seconds and then spit into a sterile container.

Subjects older than 18 years old

___ a. A blood sample, up to 30 ml (approximately 2 tablespoons) will be drawn.

___ c. A buccal smear (a light scraping of cells from your mouth with a small plastic spatula or spoon)

___ d. Skin biopsy for a small sample of non-blood cells. This would be done for research purposes only. The skin biopsy is a circular sample of skin that is taken, about the size of a pencil lead (2 mm). An anesthetic is used to prevent pain.

___ e. A mouthwash rinse sample – We ask that you rinse your mouth with water to remove any food particles and then do not drink or eat for one hour. At the end of the hour please swish approximately three teaspoons of mouthwash (mint flavored) or sterile water from side to side in your mouth, continue to swish for about 30-45 seconds and then spit into a sterile container.

Please indicate with your initials below if you give us permission to disclose the results of the genetic testing to your physician.

___ I do want my physician to be told the results of the genetic testing

___ I do not want my physician to be told the results of the genetic testing

Please indicate with your initials below if you want to know the results of the genetic testing.

___ I do want to be told the results of the genetic testing.

___ I do not want to be told the results of the genetic testing.

_____ _____
Signature of Subject Date

_____
Printed Name of Subject

If subject is a minor:

_____ _____
Signature of Parent/Legal Guardian Date

_____
Printed of Parent/Legal Guardian

Copies to: Subject
Investigator's File