

University of Washington  
**Consent/Assent Form**  
Severe Chronic Neutropenia International Registry

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Human Subjects Division

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David C. Dale, MD	Professor Department of Medicine	206-543-7215
Akiko Shimamura, MD, PhD	Associate Professor Pediatrics/Hematology-Oncology	206-667-1127
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24-Hour Emergency Telephone:	(Hematology Fellow On Call) or David C. Dale, MD (office) (pager)	206-598-6190 206-543-7215 206-986-4981

**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 called the Severe Chronic Neutropenia International Registry (Registry). 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 of 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**

We are asking you to participate in this Registry to learn more about the cause and outcome of severe chronic neutropenia (SCN). SCN is caused by several diseases and some, but not all, are inherited/genetic diseases. In all of these diseases, the levels of one type of white blood cell, the neutrophils, are very low. This condition is called severe neutropenia. People with severe neutropenia are very susceptible to infections by many types of bacteria.

The objective of the Registry is to collect information on the natural course of SCN and its response to treatment with the blood-stimulating factor called granulocyte colony stimulating factor or G-CSF (Neupogen). We also want to collect information on transplantation of blood forming cells and other treatments for severe chronic neutropenia. In addition, the Registry is studying the risk of developing of osteoporosis (thin and brittle bones), enlargement of the spleen and other changes that may affect your health. Because SCN is a rare condition, we are asking you to share your medical information with the Registry, which is involved in collecting information from

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many physicians and many patients.

## Procedures

We will also request information from your medical records: diagnosis, medical history including the history of infections, related medical problems, growth & development markers (for children this would include height, weight and milestones of physical and sexual development), types of treatment you've received for your SCN, copies of reports that describe results of previous bone marrow evaluations including special tests called cytogenetics and results of CBC (complete blood counts). We will also ask for information about the health of your immediate family members (children, sisters, brothers, parents and grandparents).

We will also ask for information about fatigue and physical abilities in a survey (questionnaire or data collection instrument). This survey is voluntary. Examples of the most sensitive questions in the survey are: how happy you are with your body and how easy or hard it is to get on and off a toilet or chair. We will send this questionnaire to you every two years.

If there is specific information that you do not want to give us, please write this in the space provided on page 5 of this form.

In addition, we will request the slides of your bone marrow tests done previously. In the future if you have more bone marrow tests, we will also ask for these slides to be sent to the Registry study. The slides are used to confirm your neutropenia diagnosis. If your referring doctor requests the slides back, they will be returned.

If your referring doctor has questions about any problems you have with your SCN, one of the researchers for the Registry study (Dr. David C. Dale, Dr. Akiko Shimamura, or Audrey Anna Bolyard) will be available to provide advice based on the information you provided us when you enrolled in the Registry study. The advice is not treatment. Your regular doctor will provide your treatment.

After joining the Registry study, you will be contacted once a year and asked to complete standard forms that will tell us about your ongoing medical condition. The information we will ask for includes whether or not you have had any infections or serious illnesses in the past year and your current health status. We will also request results of any blood tests and bone marrow tests (if done) as well as information about the dose of Neupogen you've used in the past year. Women who become pregnant while taking part in the Registry study will be asked for the outcomes of the pregnancy. We request that you give us this information to aid in our understanding of the treatment and consequences of SCN.

To help you in remembering what has happened to your health in the past year, it would be very useful if you would take notes on a calendar about your health or any illnesses you experience throughout the year. The calendar is optional and voluntary but we hope it might be helpful to you.

If you choose to participate in this study and provide the medical information requested, you will be offered access to a program to receive Neupogen at no cost to you through a pharmacy supported by Amgen, the maker of Neupogen. You can choose to participate in this program or not; it is your choice. It is important for you to understand that Amgen re-evaluates its continued support of this

program every two years so it is possible that access to continued free Neupogen could be discontinued. We will let you know if this happens. If you decide to receive Neupogen through this program, first your referring doctor must agree to write a prescription for this medication and send it to the Registry study. We will then work with you and the pharmacy to ensure you have received the first shipment of Neupogen. When you need refills the pharmacy will work directly with you and your referring doctor to arrange for this. Your referring doctor will be responsible for updating this prescription every 6 months and for monitoring your medical condition and response to Neupogen.

You do not need to participate in the Registry study to receive free Neupogen. You may contact Amgen directly to find out if you qualify for their 'Safety Net Program' which also provides Neupogen free of charge to persons of limited income without insurance. The 'Safety Net Program' contact phone number is 1-800-772-6436.

### **Risk, Stress, or Discomfort**

We are asking for your medical information, so the primary risk is to your privacy. We will store your information in locked file cabinets which are located in an office space that is locked at all times. Only the researchers will have routine access to your information. While we will make every effort to maintain the confidentiality of your information no system for protecting your information is completely secure. Researchers at other Universities working with the Registry study may have access to information about you that is coded with a study number instead of your name. Officials at the University of Washington and government offices sometimes review studies, such as this one, to make sure they are being done safely and according to local, state and federal regulations. If a review of this study takes place, your records may be examined but the reviewers will protect your privacy. The study records will not be used to put you at legal risk of harm. Your name will not be used in any published reports about this Registry study.

### **Alternatives To Taking Part In This Study**

You do not need to participate in this Registry study to obtain medical care for your SCN. You do not need to participate in order to receive Neupogen. Neupogen is approved by the FDA for the treatment of SCN and can be prescribed by your referring physician and paid for by you or your insurer. You do not need to participate in this study to receive *free* Neupogen from Amgen. You can contact the Amgen 'Safety Net Program' @ 1-800 772-6436 and, if you qualify, Amgen can provide Neupogen.

### **Benefits**

There is no direct benefit to you from participating in this Registry study but the information learned through this research may be valuable in understanding this condition and in the development of new treatments.

### **Financial Interest**

Dr. David Dale has a financial or leadership relationship with Amgen, the company which manufactures filgrastim or Neupogen, a drug use for the treatment of neutropenia. Amgen provides filgrastim free of charge to patients in the United States who are enrolled in the Severe Chronic Neutropenia International Registry (SCNIR) under a contractual agreement with the

University of Washington. Dr. Dale is the Principal Investigator for this agreement. Amgen supported development of the SCNIR and the Repository described in this consent form, but these activities are now primarily supported by a grant from the National Institutes of Health; Dr. Dale is the Principal Investigator for this grant. Dr. Dale also serves as a consultant and advisor to Amgen and receives compensation for this activity in addition to his salary from the University of Washington. This financial interest and the design of the study have been reviewed and approved by the University of Washington for possible conflict of interest. A Management Plan was developed to minimize any possible effect of this financial interest on your safety or welfare. The Plan will also protect the quality and reliability of the research.

### Other Information

If the results of the tests you provide to us do not show you have SCN, you will not be able to participate in this study and we will destroy the information you have provided. If you are eligible, agree to participate and decide you want access to the program that provides free Neupogen, it is necessary for us to give identifying information about you (such as your name, address and phone number) to the pharmacy Amgen has chosen to provide the Neupogen. This information will allow the pharmacy to ship the drug to you. You and/or your insurance company will be responsible for all other costs of your medical care, tests and treatments. You will not be paid for your participation in this study.

You may decide not to participate in the Registry study or you may withdraw at any time without penalty or loss of benefits to which you are otherwise entitled. If you choose to withdraw, we will not be able to provide you with continued access to the program providing subjects with Neupogen.

It is possible that circumstances could arise which would cause the operation of the Registry study or your participation in it to be stopped. If this happens, the reasons for stopping will be explained to you as quickly as possible. The researcher may withdraw you from the study if you are not able to provide the required medical information. We will let you know about any changes in this study.

\_\_\_\_\_  
Signature of person obtaining consent

\_\_\_\_\_  
Date

\_\_\_\_\_  
Printed name of person obtaining consent

\_\_\_\_\_  
Date

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UW Human Subjects  
Review Committee

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.

**Medical records:**

Please indicate with your initials if you have medical information you do not wish to share with the Neutropenia Study.

\_\_\_\_\_ I release my medical information to the Neutropenia Study.

\_\_\_\_\_ I release my medical information to the Neutropenia Study **with the exclusions listed below:**

\_\_\_\_\_  
\_\_\_\_\_  
\_\_\_\_\_

\_\_\_\_\_  
Signature of Subject

\_\_\_\_\_  
Date

\_\_\_\_\_  
Printed Name of Subject

When a subject is a minor:

\_\_\_\_\_  
Signature of Parent/Legal Guardian

\_\_\_\_\_  
Date

\_\_\_\_\_  
Printed of Parent/Legal Guardian

Copies to: Subject  
Investigator's file

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**ASSENT FORM**  
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Investigator's Statement

**Purpose and Benefits**

We are asking you to be in a science study. We will tell you about the study and then ask you if you want to be in the study.

We are asking you to be in the study because you have a disease called severe chronic neutropenia. This means the amount of cells in your blood called neutrophils are very low. Blood is made up of different types of cells. A neutrophil is one type of cell in the blood. Neutrophils help your body to fight infections. When your blood does not have enough neutrophils, you may become sick from infections. Not many people have severe chronic neutropenia. This condition is very rare.

A medical registry is a collection of information about patients with a certain medical condition. This registry will collect information about neutropenia from people all over the world. In the science study, we want to see if your blood is different from other people who do not have this problem. We hope information about you will help us learn more about neutropenia.

The Registry collects information about neutropenia from people all over the world. We collect information about things like blood tests, bone marrow tests, whether you were sick, what medicines you are taking, how you are growing and developing, such as your height, your weight and if your body has started to change into a young adult. It is very helpful to the study if you share this information with us.

**Procedures**

You and your parents will send us information about your neutropenia and will fill out a form once a year as long as you are in the study to let us know how you are doing. This information will be

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kept in locked files and on a computer in the locked Registry office.

Every two years you and your parents will be asked to fill out another form. We will ask for information about whether or not you are tired and how easy or hard it is for you to do different activities. This form is voluntary.

You or your parents may be asked to fill out a calendar about your neutropenia. The calendar may help you and your parents remember the events of the last year. This calendar is meant to be an aid for you and your parents. This is voluntary; you do not have to give us this information to remain in the Registry. You and your parents may use this calendar to help fill out the yearly form.

### **Risks, Stress, and Discomfort**

The calendar form will only take a few minutes once a day or once a week to fill out. You do not have to fill out the calendar form.

### **Financial Interest**

Dr. David Dale has a financial or leadership relationship with Amgen, the company which manufactures filgrastim or Neupogen, a drug use for the treatment of neutropenia. Amgen provides filgrastim free of charge to patients in the United States who are enrolled in the Severe Chronic Neutropenia International Registry (SCNIR) under a contractual agreement with the University of Washington. Dr. Dale is the Principal Investigator for this agreement. Amgen supported development of the SCNIR and the Repository described in this consent form, but these activities are now primarily supported by a grant from the National Institutes of Health; Dr. Dale is the Principal Investigator for this grant. Dr. Dale also serves as a consultant and advisor to Amgen and receives compensation for this activity in addition to his salary from the University of Washington. This financial interest and the design of the study have been reviewed and approved by the University of Washington for possible conflict of interest. A Management Plan was developed to minimize any possible effect of this financial interest on your safety or welfare. The Plan will also protect the quality and reliability of the research.

### **Other Information**

We won't tell anyone you took part in this study. We will not give your name to anyone else. Sometimes, to help other doctors learn about neutropenia, we will tell the doctors about your neutropenia and medical condition, however we will not tell them your name. We will use your information in future research and researchers (people who do research on neutropenia) from other institutions will have access to the information, but not to your name. You don't have to take part in this study if you don't want to. We will give you a copy of this paper to keep.

\_\_\_\_\_  
Signature of person obtaining consent

\_\_\_\_\_  
Date

\_\_\_\_\_  
Printed name of person obtaining consent

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Subject's Statement

This research study has been explained to me. I agree to take part in this study. I have had a chance to ask questions. If I have more questions, I know I can ask Dr. David C. Dale or Audrey Anna Bolyard.

\_\_\_\_\_  
Signature of Subject

\_\_\_\_\_  
Date

\_\_\_\_\_  
Printed Name of Subject

Copies to: Subject  
Investigator's file

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Verbal Script  
Severe Chronic Neutropenia International Registry

(Children approximately 2 years of age or older, under 7 years)

"Hi. My name is \_\_\_\_\_. I try to help people with problems with their blood. I will write down information about your neutropenia and your health on paper. It is like a book. I would like to ask you and your parents to send copies of information about your neutropenia and health to the Registry. Will that be okay?"

Copies to: Subject  
Investigator's file

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For the Use of Patient Health Information for Research

Research Title: Severe Chronic Neutropenia International Registry  
Lead researcher: David C. Dale, MD  
Institution of lead researcher: University of Washington

**A. Purpose of this form**

The purpose of this form is to give your permission to the research team to obtain and use your patient health information. Your patient information will be used to do the research named above.

*This document is also used for parents to provide permission to obtain the patient information of their minor children, and for legally-authorized representatives of subjects (such as an appropriate family member) to provide permission to obtain patient information of individuals who are not capable themselves of providing permission. In such cases, the terms "you" and "your patient information" refer to the subject rather than the person providing permission.*

State and federal privacy laws protect your patient information. These laws say that, in most cases, your health care provider can release your identifiable patient information to the research team only if you give permission by signing this form.

You do not have to sign this permission form. If you do not, you will not be allowed to join the research study. Your decision to not sign this permission will not affect any other treatment, health care, enrollment in health plans or eligibility for benefits.

**B. The patient information that will be obtained and used**

"Patient information" means the health information in your medical or other healthcare records. It also includes information in your records that can identify you. For example, it can include your name, address, phone number, birthdate, and medical record number.

1. Location of patient information

By signing this form you are giving permission to the following organization(s) to disclose your patient information for this research.

Name of health care organization(s) or provider(s):

\_\_\_\_\_

2. Patient information that will be released for research use

This permission is for the health care provided to you during the following time period:

From the time of your enrollment into the study through July 31, 2014.

The specific information that will be released and used for this research is described below:

- All records for which you have given permission to be sent to us by your physician

### 3. Use of the UW Clinical Research Center (CRC)

Some of the research procedures may occur at the UW Clinical Research Center (CRC). In the unlikely event something happens to you that requires treatment while you are at the CRC, information about the event and treatment will also be released to the researcher. Examples: fainting during a blood draw or stumbling while entering the blood draw area.

### C. How your patient information will be used

The researcher will use your patient information only in the ways that are described in the research consent form that you sign and as described here.

The research consent form describes who will have access to your information. It also describes how your information will be protected. You can ask questions about what the research team will do with your information and how they will protect it.

The privacy laws do not always require the receiver of your information to keep your information confidential. After your information has been given to others, there is a risk that it could be shared without your permission.

### D. Expiration

This permission for the researchers to obtain your patient information:

ends on July 31, 2014.

### E. Canceling your permission

You may change your mind at any time. To take back your permission, you must send your **written** request to:

Audrey Anna Bolyard  
Severe Chronic Neutropenia International Registry  
University District Bldg  
1107 NE 45<sup>th</sup> St., Suite 345  
Seattle, WA 98105

If you take back your permission, the research team may still keep and use any patient information about you that they already have. But they can't obtain more health information about you for this research unless it is required by a federal agency that is monitoring the research.

If you take back your permission, you will need to leave the research study. This means that you would not have any more research treatments or tests. Changing your mind will not affect any other treatment, payment, health care, enrollment in health plans or eligibility for benefits.

### F. Giving permission

You give your permission to release your information by signing this form.

To release the specific information listed below, you need to also write your initials next to the type of information. This is your specific permission for release of this information, which is required by Federal and state laws. The federal rules bar any use of the information to criminally investigate or prosecute any alcohol or drug abuse patient.

- \_\_\_\_\_ Sexually transmitted disease
- \_\_\_\_\_ AIDS or HIV
- \_\_\_\_\_ Behavioral or mental health/illness, including psychotherapy notes
- \_\_\_\_\_ Drug or alcohol abuse, diagnosis, or treatment

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Printed Name of Research Subject

Birthdate

---

Signature of Research Subject

Date of signature

---

Printed Name of Person Authorized to Give Permission

---

Signature of Person Authorized to Give Permission

Date of signature

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Relationship to Subject and Description of Authority

(Examples: parent of a young child; sister of an individual who is in a coma; researcher who signs for a subject who is unable to physically sign the authorization but was observed by the researcher to read and otherwise agree to the authorization.)

You will receive a copy of this signed form. Please keep it with your personal records.