

JUL 08 2009

APPROVED

**CONSENT FORM – For Parents and Patients 18 and older
ASSENT FORM – For Patients 14 – 17 years of age**

**Consent to Participate in a National Registry of Patients
with Primary Immune Deficiency Diseases (PIDD)**

Investigators:

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Emergency (24 hours): 1-866-987-2000 Seattle Children's Hospital Switchboard: ask for the Immunology attending physician on call.

Investigator's Statement:

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

Introduction

We are asking you to take part in a research study. Taking part in this research is voluntary. Please take the time you need to make your decision and discuss with family and friends. You do not have to take part in this study if you do not want to. If you take part, you can leave the study at any time.

Why is this research study being done?

You have a rare Primary Immune Deficiency Disorder (PIDD). PIDDs are a group of diseases that make you vulnerable to infections. People with certain types of PIDDs have an increased risk of autoimmune problems (where your own immune system attacks your body) and certain cancers. The U.S. Immunodeficiency Network (USIDnet) wants to build a National Registry of individuals with certain types of PIDD. A "registry" is a list of basic information, both clinical and laboratory, about individuals who have a certain disease or condition in common.

The National Registry for PIDD will include the following diseases:

- Severe Combined Immune Deficiency (SCID)

- Agammaglobulinemia with either X-Linked (XLA) or autosomal recessive inheritance
- Common Variable Immune Deficiency (CVID)
- Hyper IgM syndrome (HlgM)
- Wiskott-Aldrich Syndrome (WAS)
- Chronic Granulomatous Disease (CGD)
- Leukocyte Adhesion Deficiency (LAD)
- DiGeorge Syndrome (DGS)
- Anhydrotic Ectodermal Dysplasia (NEMO)
- Complement deficiencies

A National Registry of these rare diseases will provide important information such as:

- how common the disorders are
- the differences in how one disorder affects different people
- the amount of time it takes to be diagnosed
- what complications are found with the diseases
- how effective different therapies are
- what the quality of life is in affected people
- what the long term outcome is in patients

Because these are very rare diseases, no single medical center has the number of patients needed to answer these questions. Because this Registry is ongoing and new information from participating individuals will be recorded over time, it is necessary to link the clinical and laboratory data with identifiers such as name, date of birth, sex, race/ethnic group (Option 1). In addition, we will collect the type of complications that has occurred since the diagnosis was established and the outcomes of various treatments used.

Once this information is entered into the Registry, you will have a unique number assigned to you that will separate your identifying information from the Registry data. Only the data manager and your physician will be able to connect this number with you. Thus, anyone authorized to analyze data collected in the Registry will not be able to link the clinical and laboratory data with you. You will not be contacted by anyone unless you authorize it. If a new study about your disease comes up, your personal doctor will be notified. He or she can share this information with you to find out if you are interested in participating or not. Alternatively, you may elect to be contacted directly by the Registry to determine your interest in participation.

Your physician will be asked to update the Registry with your most recent information when you make regular clinic visits. Over 150 different primary immune deficiency disorders have now been described. As the Registry expands, other PIDDs may be added to the list of 10 disorders mentioned above. You qualify for inclusion in this Registry because you have one of these 10 disorders. We are asking your permission to submit the results of your immunologic studies and your basic clinical information to this National Database using your personal identifiers. We also want to periodically update the Registry when you come in for clinic visits.

If you choose not to provide identifiers such as name and date of birth, we will limit the personal data to the name of the referring physician, the year of your birth, the state of birth, the state of residency,

gender, race/ethnicity, and diagnosis, including the molecular defect (Option 2). This minimal information will keep the details about your medical problems confidential, as only your personal physician will be able to identify you.

What are the benefits to taking part in the study?

There may or may not be a direct benefit for you if you participate in this study. Your participation may help doctors to better understand your medical condition and may lead to earlier diagnosis and to better treatments. Submitting your data into the Registry will also advance medical knowledge in general, so other patients may benefit.

What is involved in the study?

By building a National Registry of patients with PIDD, we are trying to understand how many people are affected with these rare diseases and how we might better diagnose and treat them.

If you agree to provide identifying information (Option 1) it will be easy to collect follow-up data as to the course of the disease, complications, response to treatment and quality of life. Under these options, your medical information will be assigned a code number which will be included in the Registry Database. Your personal identification information will be kept in a separate location. Only the Registry manager and the referring physician will know the code number. The doctors and scientists studying these illnesses using the Registry will have access to your medical information as described below, but they cannot link to your identity using the code number.

Doctors and scientists will only have access to information in the Registry by getting permission from the Registry Review Committee, a group of experts in PIDD research. Even then, only anonymous information will be shared. Your personal identity will never be revealed to these doctors/scientists and your name will never appear in scientific reports.

It is possible that in certain circumstances, when a new test or new treatment is being developed, doctors or scientists will be able to request the name of physicians who treat patients with certain immune defects. If this request is approved by the Registry Review Committee, your physician may be notified that a new test or treatment is available. If your physician thinks that this is appropriate, he/she will let you know of this development and you will have the option to decide if you are interested or not. However, your physician will not reveal your name or your identity unless this is your wish.

Under Option 2, we will only submit the name of your physician, the year of your birth, state of birth, state of residency, gender, race/ethnicity and diagnosis to the Registry. Your identity will not be revealed to the Registry. Under this scenario, if the referring physician remains in contact with you, he/she will be able to submit follow-up data to the Registry without revealing your identity.

Transfer of pre-transplant data from USIDnet Registry into the CIBMTR registry:

If you have an immunodeficiency that can be treated with bone marrow stem cell transplantation, we would like to obtain your permission to transfer the data collected in the National Registry of Patients with PIDD, organized by the USIDnet) Registry to a separate registry for patients undergoing bone marrow transplantation called Center for International Blood and Marrow Transplant Research (CIBMTR). The data collected before transplantation in the USIDnet Registry are important for those doctors who perform the bone marrow/stem cell transplantation, therefore we ask you to allow us to transfer this data set into the registry of the CIBMTR. During and after the stem cell transplantation, the transplant doctors will collect and submit clinical data, laboratory details and the status of the transplant to the CIBMTR-Registry. Once the bone marrow transplantation procedure has been completed, you most likely will be followed by your immunologists, who had earlier referred you to the bone marrow transplant team. In order for the immunologists to be completely informed about the details of your bone marrow transplantation, the data collected in the CIBMTR-Registry will be transferred back to the USIDnet. This will allow a seamless transfer of information first from USIDnet National Registry to the CIBMTR-Registry and then from CIBMTR-Registry to the USIDnet National Registry, guaranteeing that your physicians have access to all pertinent information.

I agree that data can be transferred from the USIDnet National Registry to the CIBMTR-Registry and after transplantation from the CIBMTR-Registry to the USIDnet National Registry.

I do not wish to have my data exchanged with the CIBMTR-Registry.

What are the risks of taking part in this study?

There are no major risks for your participation in this Registry. Your name and other personal identifiers will be replaced with a code number in the Registry. The doctors or scientists using the Registry Database will not know your identity. There could be a small chance that the data entry persons at the Registry could identify you should you select option 1 or could make a guess about your name from the minimal information provided under option 2. They are required to keep your information confidential. As mentioned before, under option 1 your identifying information will be kept separately from your medical information in the Registry and will be identified only by a code number. Your personal identification will not be available to doctors/scientists using the Registry. While there is the possibility that your confidential information could become known, the people administering the Registry are taking appropriate actions to minimize the risk of this happening.

As outlined above, we will make every effort to keep your personal information confidential. Your identity will be kept confidential from any publications resulting from this study. The information obtained will be kept confidential to extent permitted by law. However, your personal medical record may be reviewed by government agencies, such as the Food and Drug Administration or the Department of Health and Human Services (the agency sponsoring this research), or by individuals who are authorized to monitor or audit the project, or by the Institutional Review Board (IRB), the committee that oversees all research in humans at any institution participating in the Registry, if required by applicable laws or regulations.

Can I change my mind about submitting my medical data to the Registry?

After your medical information is submitted, it will be pooled with the data of many other subjects. If you select Option 1 (e.g. submitting identifying data such as name, date of birth, address, etc.) it will be possible to remove your identifying information from the Registry records at any time. It is possible to remove your data from the Registry, since it remains linked to you by a unique code. If you elect Option 2, it will be more difficult to later remove your own data from the Registry. It would require that the physician who submitted the data to the Registry identifies you based on diagnosis, year of birth, state of birth and state of current residency.

The Registry is voluntary. If you decide not to participate, this will not affect your ability to receive medical care or to receive any benefits to which you are otherwise entitled.

What are the costs associated with having data entered into the National Registry?

As the Registry is supported by funds from the National Institutes of Health, there will be no cost to you or your family.

Will I be paid to take part in the study?

You will not be paid to take part in this research study.

What will happen to the data collected in the National Registry?

This is an ongoing study of patients with primary immune deficiency disorders. The Registry will continue as long as it is supported by the National Institutes of Health. Experts in the field, including physicians and scientists, will have the opportunity to analyze the data, to use information from the Registry to design new clinical trials, and to publish these results in medical journals, but they will not have access to your identifying information. No information will be published that directly identifies you. The data collected are owned by the National Institutes of Health, a government agency.

What are your rights as a research participant?

Taking part in research is always voluntary. You may decide not to take part. If you choose to take part, you may withdraw at any time. Your decision will not affect your medical care. There are no penalties or loss of benefits if you choose not to take part or to withdraw from the study after you have started to take part.

A signed copy of this consent form will be given to you.

Who do you call if you have questions or problems?

If you have any questions, at any time, about this Registry, or want to discuss any possible Registry-related injuries, please contact Hans D. Ochs, MD, or any of the other healthcare professionals listed

on page one of this consent form at Seattle Children's Hospital by dialing (206) 987-7450 or 1-(866)-987-2000.

If you have questions about your (your child's) rights as a research participant, please call Seattle Children's Institutional Review Board (IRB). The IRB is responsible for protecting the rights of children and families taking part in research. The IRB may be reached at (206) 987-7804.

Printed Name of Person Obtaining Consent

Signature of Person Obtaining Consent Date

Research Participant's Statement:

The research described above has been explained to me. I voluntarily consent to participate (allow my child to participate) in the PIDD Registry, under the supervision of the Registry Review Committee of the USIDnet Research Consortium. I wish to participate following the conditions outlined in

OPTION 1 (), in which my identity will be kept in a separate location by the Registry and will be linked to my personal medical information by assigned code number

OR

OPTION 2 (), in which no record of my personal identity will be kept by the Registry

I have had an opportunity to ask questions. I understand that future questions I may have about the research or about my (my child's) rights will be answered by the persons listed on page one of this form.

*** If the child to be involved in this research study is a foster child or ward of the state, please notify the researcher or the staff member obtaining your consent.**

Name of Research Participant

Signature of Research Participant if 14 or older

Date

Signature of Parent/Legal Guardian

Date

Signed original of this form must be filed in:

Researchers' file

Copies of signed form provided to:

Research Participant/Parent

And, if participant is a Seattle Children's patient:

Seattle Children's Medical Record

Permission to Use, Create and Share Health Information for Research

**Research Study Title: A national registry of patients with primary immunodeficiency diseases
IRB Study #: 12601**

The federal Privacy Rule protects your/your child's health information. The Privacy Rule is part of the Health Insurance Portability and Accountability Act (HIPAA).

If you/your child agree to take part in this research study (named above), the researchers may use, create or share your/your child's health information as part of the research. The researchers will do so **only** if you give permission to use, create or share your/your child's health information as part of the research. This form gives you information to help you decide if you will give such permission. **Please read this form carefully.** After reading this form, you can refuse to sign this form.

What does "health information" include? It includes:

- Name Address Social Security Number Medical and/or birth history Demographic information
- Results of physical exams Results of laboratory and/or radiology tests
- Interview and/or focus group data Survey and/or questionnaire data
- Results of behavioral tests Information related to your health condition
- Information in your medical record relevant to this study Other (please specify) * _____

* If using a translated HIPAA Form, this information must also be translated

What the researchers may do with health information

Researchers may create new health information about you/your child during the study. Researchers may use health information in your/your child's records.

Researchers may also share health information about you/your child collected during the study with the following:

1. The sponsor of this study and its representatives. Sponsor Name:
2. Researchers at other centers taking part in this research study.
Name(s) of other center(s):
3. Government agencies, ethics review boards, data and safety monitoring boards, and others responsible for watching over the safety, effectiveness, and conduct of the research.
4. Your health care insurance company if it is paying for care provided as part of the research study.
5. Other health care providers involved in your/your child's care.
6. National Institutes of Health and its grant holders for the purpose of research administrative activities (e.g., tracking overall research activity).
7. Others, as provided by law. **USIDnet**

The Privacy Rule applies to doctors, hospitals and other health care providers. Some of the groups listed above are not required to follow the Privacy Rule and may share your/your child's information with others, if other laws allow. However, other privacy protections may still apply.

Research Records

You may look at or copy the information that may be used or disclosed. However, for certain types of research studies, some of the research records may not be available to you/your child while the study is going on. This does not affect your right to see what is in your/your child's medical (hospital) records.

The researchers may publish or present the research findings. You/your child will not be identified in any findings that are published or presented.

The federal Privacy Rule does not apply to health information that is not identified in any way. The researchers may decide to remove any information that could identify you/your child. If they do this, the information may be used and shared by the researchers and the sponsor as the law allows. This may include use in other research studies.

Permissions to Take Part in Research

If you agree to take part or allow your child to take part in the research, you will be asked to sign a **research consent form**. The research consent form gives you details about the research. The consent form describes the risks and benefits of the research. It explains the purpose of the study, what will happen and other important information for you to know.

To be in this research study, you must also sign this permission form (Permission to Use, Create and Share Health Information for Research). If you do not want to sign this permission form, this will not affect the care and treatment you or your child receive.

How Long does the Permission Last? What if You Change Your Mind?

- This permission is valid until the end of the research study;
or
 This permission will not expire, because this is a research database or repository study (i.e. specimens and/or data are stored permanently).

Except for the research database and repository studies, your/your child's information will be destroyed or any personal identification will be removed at the end of the research study. If you change your mind and want to cancel your permission, please let us know in writing. Write to Principal Investigator (PI)/Researcher:

Hans D. Ochs, M.D.
Professor of Pediatrics
Jeffrey Modell Chair of Pediatric Immunology Research
University of Washington School of Medicine

Seattle Children's Hospital
Seattle Children's Research Institute
1900 Ninth Avenue, C9S-7
Seattle, WA 98101
phone: 206-987-7450
fax: 206-987-7310

If you cancel your permission and you/your child are a patient at Seattle Children's, please send a copy of your letter to:

Director of Health Information and Privacy, Health Information Management, A-4902, Seattle Children's Hospital, 4800 Sand Point Way NE, Seattle, WA 98105-0371.

If you cancel your permission, no other health information about you/your child will be collected for this research. However, the health information that was received with your permission may be shared or used. For example, researchers may need to use or share this information:

- for safety reasons;
- to verify the research data;
- if required by law.

If you agree to take part or allow your child to take part, you will be given a copy of this permission form after you have signed it.

Permission

I agree to the use, creation, and sharing of my or my child's health information for purposes of this research study (named on page 1). For Seattle Children's patients, your medical record # will be recorded on this form and used to place a copy of this form in your medical record.

_____ Printed Name of Participant	_____ Signature of Participant (if 18 years or Older)	_____ Date
_____ Printed Name of Participant's Parent or Legal Representative	_____ Signature of Research Participant's Parent or Legal Representative (if younger than 18 years)	_____ Date

Researcher Obtaining Authorization

_____ Printed Name of Research Team Member*	_____ Signature of Research Team Member	_____ Date
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***INSTRUCTIONS TO RESEARCHER**

1. File signed <u>original</u> of this form in Research File	<input type="checkbox"/>
2. Provide <u>copy</u> of signed form to Research Participant/Parent	<input type="checkbox"/>
<u>For Seattle Children's Patients</u>	
3. Complete or attach patient label:	<input type="checkbox"/>
<div style="border: 1px dotted black; padding: 10px; margin: 5px 0;"> Participant's Medical Record # _____ Participant's Date of Birth _____ / _____ / _____ </div>	
4. Send <u>copy</u> of the signed form to Health Information Filing: Mailstop A-4902	<input type="checkbox"/>