

CHILDREN'S NATIONAL MEDICAL CENTER

Center for Genetic Medicine, Children's Research Institute
111 Michigan Avenue, NW
Washington, DC 20010
(202) 476 6011

ASSENT (AGES 12 to 18) TO PARTICIPATE IN A CLINICAL RESEARCH STUDY

Family Member of Patient with a Leukodystrophy

TITLE OF STUDY: New Diagnostic Approaches In Leukodystrophy

PRINCIPAL INVESTIGATOR: Adeline Vanderver, MD; Department of Neurology

INTRODUCTION: We would like to invite you to be part of a research study at Children's National Medical Center. Before you decide if you would like to participate, we want you to know why we are doing the study. We also want you to know about any risks (anything unexpected that might happen) and what you will be expected to do in the study. You can only be in the study if your parent(s) agree(s).

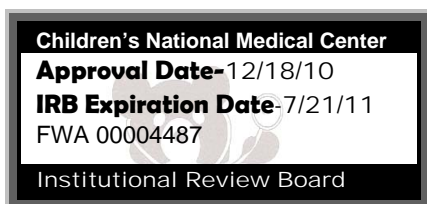
This form gives you information about the study. Your doctor or a research staff member will talk to you about the study and answer any questions you have. We encourage you to discuss this study with your family before making your decision. We will ask you to sign this form to show that you understand the study. We will give you a copy of this form to keep. It is important that you know:

- You do not have to join the study;
- You may change your mind and stop being in the study any time you want and no one will mind.
- If we make any important change to the study we will tell you about it and make sure you still want to be in the study.

A. WHAT IS THE REASON FOR THE STUDY?

Your doctor thinks that your family member has a leukodystrophy. Leukodystrophy is a disease that affects the white matter. The white matter is the area of the brain through which messages are sent. A substance called myelin allows messages to move quickly through this area and causes the area to look white in color, which is why it is called white matter. Leukodystrophies can have many causes, some known and some yet to be recognized. If we think we can learn about leukodystrophies with your help and the

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help of others like you, we may ask your doctor to send us a blood or saliva sample from you so that we can do research related to your family member's condition.

This study will help us learn about the genes involved in Leukodystrophies. Genes are the recipes for how our body works and the DNA are the words/letters that make up this recipe. Family members sometimes share the same genes, and sometimes have different genes. Understanding what genes you and your sibling share and do not share may help us understand why your family members is sick, but you are not. This research may help to find what causes leukodystrophies as well as find new tests to diagnose them.

B. WHAT WILL HAPPEN IN THE STUDY?

If you participate, your doctor will send us a sample of your blood or saliva. This study uses experiments to look at genes and proteins. Genes carry the instructions for how everything in our body works. Proteins are created from the instructions in the genes and carry that message to the body. When we look at your DNA, it is possible that we will find other genetic changes that could affect you or your family members, unrelated to your family member's leukodystrophy. If we find genetic changes that are relevant to your health, and that have a treatment, we will tell you or your family members know.

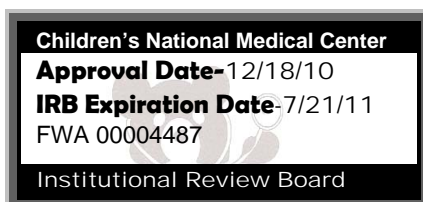
We will keep your sample until this study is over. If you decide at anytime that you do not want to be in this study anymore, you can call the principal investigator, Dr. Vanderver, and she will take you out of the study. That means that she will destroy any samples that you have donated and she will take your name out of the database.

C. WHAT POSSIBLE UNEXPECTED THINGS COULD HAPPEN?

The risks associated with this study are limited to those related to confidentiality. The risks associated with obtaining the samples to find out your leukodystrophy should be explained to you by the doctor obtaining the samples. All the potential risks explained below should be explained to you in detail by your doctor.

1. For tests on your DNA we need one sample of blood (about 2 teaspoons). The needle puncture may cause some bleeding, mild pain or infection. However, the procedure is to be performed by individuals trained and experienced in obtaining blood samples so as to minimize these risks.
2. There is a chance that you may feel stress from participating in our research. Knowing that your family member has a leukodystrophy can lead to feelings of

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anxiety or sadness. Stress may also occur in knowing that your family member has a genetic condition, but that the doctors don't have a cure or treatment. We have a genetic counselor who can answer questions you have about the research. If you are upset, you should talk to your doctor about this. If you are unsure if you want to be in this research study, the genetic counselor can help you decide if you want to participate.

3. Researchers may learn about your genes. Unless this information is relevant to your health and wellbeing, this information will not be shared with you as part of this research protocol. If you have questions about this, you may discuss this with the genetic counselor employed by Dr. Vanderver's laboratory or with Dr. Vanderver.

D. WHAT POSSIBLE GOOD THINGS COULD HAPPEN?

By doing research on blood and saliva samples from the family members of patients with a leukodystrophy, Dr. Vanderver's laboratory can identify new causes for health problems and a better understanding of why the disease happens. By doing this research, we expect to find gene and protein changes that cause disease and we hope to learn more about their effect on the body. This research helps us to create accurate tests and may help our group and others to find medicines or other therapies to treat people with genetic conditions. If we find any results through our research that might help you directly, we will let your doctor know so that he (she) can tell you.

E. WHAT OTHER CHOICES DO YOU HAVE IF YOU DO NOT WANT TO BE IN THE STUDY?

If you decide not to participate it will not affect your health care in any way.

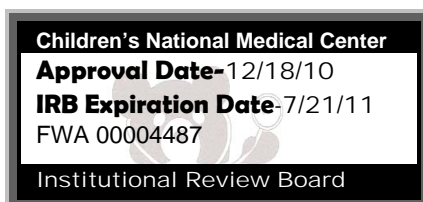
F. HOW WILL WE KEEP YOUR RECORDS PRIVATE?

We will keep the records of this study confidential. Only the people working on the study will know your name. They will keep this information in case we have to find you later to let you know of any new information that may affect your health.

ASSENT

By signing this form, you agree that you have talked to your doctor about the study and understand it, and want to be in the study. You also agree that you have been told about the risks (unexpected things) and benefits (good things) of the study, and about other

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choices. You may stop being in the study at any time and no one will mind and nothing will change about your medical care other than not being in the study. By signing this form, you agree to participate in the following aspects of this research:

1. Collection of clinical information (medical records, MRI, clinical exam)
2. Collection of blood for gene and other studies
3. After use for this study, my blood or saliva samples will be stored to be used in future studies related to leukodystrophies only.

Please call the Principal Investigator, Dr. Adeline Vanderver, at 202-476-6230, if you have any questions.

Printed Name of Participant: _____

Medical Record Number: _____

Signature of Participant: _____

Witness (to signature): _____ **Date:** _____
(may be investigator)

Translator's Signature (if, applicable): _____ **Date:** _____
Language: _____

AFFIDAVIT OF PERSON OBTAINING ASSENT: I certify that I have explained to the above individual(s) the nature and purpose of the study, potential benefits, and possible risks associated with participation in this study. I have answered any questions that have been raised.

Printed Name of Individual Obtaining Consent: _____

Title: _____ Signature: _____ Date: _____

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