

CONSENT FORM TO PARTICIPATE IN A RESEARCH STUDY

INVESTIGATOR'S NAME: MARTIN L. KATZ, PH.D.

PROJECT # 1202732

Study Title: Assessment of Blood BioMarkers in Late Infantile Neuronal Ceroid Lipofuscinosis

INTRODUCTION

This consent may contain words that you do not understand. Please ask the investigator or the study staff to explain any words or information that you do not clearly understand.

This is a research study. Research studies include only people who choose to participate. As a study participant you have the right to know about the procedures that will be used in this research study so that you can make the decision whether or not to participate. The information presented here is simply an effort to make you better informed so that you may give or withhold your consent to participate in this research study.

Please take your time to make your decision and discuss it with your family and friends.

You are being asked to take part in this study because you have late infantile neuronal ceroid lipofuscinosis (LINCL), are related to someone with LINCL, or do have this disease and are unrelated to anyone with this disease but are willing to serve as a healthy control. Information about LINCL can be found at <http://www.bdsra.org/what-is-batten-disease/about-batten-disease/>.

This study is being sponsored by the University of Missouri.

In order to participate in this study, it will be necessary to give your written consent.

WHY IS THIS STUDY BEING DONE?

The purpose of this study is to determine whether the concentrations of any chemicals in the blood are abnormal in people with LINCL.

This research is being done because we are developing a possible treatment for LINCL and we need to identify disease-related abnormalities in the blood so that in the future we can determine whether these abnormalities are corrected by treatments for the disease.

HOW MANY PEOPLE WILL TAKE PART IN THE STUDY?

About 100 people will take part in this study nationwide.

WHAT IS INVOLVED IN THE STUDY?

If you take part in this study, you will be asked to answer the questions included in this form and to donate approximately 10 ml (two tubes) of blood to be drawn from an arm vein. The blood can be drawn by any individual licensed to perform this procedure. The blood will either be drawn at the annual meeting of the Batten Disease Support and Research Association (BDSRA) or at a location and time that is convenient for you. If the samples are drawn at the BDSRA meeting, Prof. Katz will take them with him back to his laboratory. If the samples are collected elsewhere, they will be shipped to Prof. Katz along with a completed copy of this form. You can request instructions for shipping samples by contacting Prof. Katz via e-mail at katzm@health.missouri.edu.

HOW LONG WILL I BE IN THE STUDY?

We think you will only need to donate blood one time for this study unless you are affected with LINCL. For affected individuals we may request blood donations no more frequently than one time per year for up to four years after the first sample was taken.

The investigator and/or your doctor may decide to take you off this study if you show an adverse reaction to having a blood sample taken or if funding for this project is no longer available.

You can stop participating at any time. Your decision to withdraw from the study will not affect in any way your medical care and/or benefits.

WHAT ARE THE RISKS OF THE STUDY?

While on the study, you are at risk for developing a bruise around the site on your arm around the site where the needle is inserted to collect the blood sample. This is rare when blood is drawn by trained personnel. If such a bruise develops, it will resolve without treatment within a few weeks and is rarely painful. There is also a risk of excessive bleeding if you suffer from a blood clotting disorder or are taking medication that inhibits blood clotting. If either of these conditions applies to you, it would be best if you not donate blood for this study.

For the reasons stated above the person who draws your blood will observe you closely during the blood draw and, if you have any worrisome symptoms or symptoms that the investigator or his associates have described to you, notify the investigator immediately. Investigator's telephone number is 573-882-8480 For more information about risks and side effects, ask the investigator.

ARE THERE BENEFITS TO TAKING PART IN THE STUDY?

If you agree to take part in this study, there may or may not be direct medical benefit to you. You may expect to benefit from taking part in this research to the extent that you are contributing to medical knowledge. We hope the information learned from this study will benefit patients with LINCL in the future.

WHAT OTHER OPTIONS ARE THERE?

An alternative is to not participate in this research study.

WHAT ABOUT CONFIDENTIALITY?

A copy of this consent will be placed in the research records of Prof. Katz. Only Prof. Katz or his technical staff will have access to personal information provided unless otherwise mandated by law. Anyone accessing your record will be able to view the document and see that you have agreed to participate in the study. Information that does not become part of your medical record will be stored in the investigator's file and identified by a code number only. The code key connecting your name to specific information about you will be kept in a separate, secure location. Information contained in your records may not be given to anyone unaffiliated with the study in a form that could identify you without your written consent, except as required by law. If the investigator conducting this study is not your primary, or regular doctor, he must obtain your permission before contacting your regular doctor for information about your past medical history or to inform them that you are in this trial.

It is possible that your medical and/or research record, including sensitive information and/or identifying information, may be inspected and/or copied by the study sponsor (and/or its agent), the Food and Drug Administration (FDA), federal or state government agencies, or hospital accrediting agencies, in the course of carrying out their duties. If your record is inspected or copied by the study sponsor (and/or its agents), or by any of these agencies, the University of Missouri will use reasonable efforts to protect your privacy and the confidentiality of your medical information.

The results of this study may be published in a medical book or journal or used for teaching purposes. However, your name or other identifying information will not be used in any publication or teaching materials without your specific permission.

WHAT ARE THE COSTS?

You will not be charged for blood draws that are part of this research study. You or your insurance company will, however, be charged for any portion of your care that is considered standard care. Professor Katz or his laboratory staff will provide blood sample donors with a University of Missouri FedEx account number that can be billed directly for the costs of shipping any samples to the laboratory at the University of Missouri. In the cases where the facilities doing the blood collections and sample shipping charge for their services, the charges for these services may be billed to the University of Missouri via invoices sent to Prof. Katz.

You or your insurance company will be charged for continuing medical care and/or hospitalization.

WILL I BE PAID FOR PARTICIPATING IN THE STUDY?

You will receive no payment for taking part in this study.

WHAT IF I AM INJURED?

It is not the policy of the University of Missouri to compensate human subjects in the event the research results in injury. The University of Missouri, in fulfilling its public responsibility, has provided medical, professional and general liability insurance coverage for any injury in the event such injury is caused by the negligence of the University of Missouri, its faculty and staff. The University of Missouri also will provide, within the limitations of the laws of the State of Missouri, facilities and medical attention to subjects who suffer injuries while participating in the research projects of the University of Missouri. In the event you have suffered injury as the result of participation in this research program, you are to contact the Risk Management Officer, telephone number (573) 882-1181, at the Health Sciences Center, who can review the matter and provide further information. This statement is not to be construed as an admission of liability.

WHAT ARE MY RIGHTS AS A PARTICIPANT?

Participation in this study is voluntary. You do not have to participate in this study. Your present or future care will not be affected should you choose not to participate. If you decide to participate, you can change your mind and drop out of the study at any time without affecting your present or future care. Leaving the study will not result in any penalty or loss of benefits to which you are entitled. In addition, the investigator of this study may decide to end your participation in this study at any time after he has explained the reasons for doing so and has helped arrange for your continued care by your own doctor, if needed.

You will be informed of any significant new findings discovered during the course of this study that might influence your health, welfare, or willingness to continue participation in this study.

WHOM DO I CALL IF I HAVE QUESTIONS OR PROBLEMS?

If you have any questions regarding your rights as a participant in this research and/or concerns about the study, or if you feel under any pressure to enroll or to continue to participate in this study, you may contact the University of Missouri Health Sciences Institutional Review Board (which is a group of people who review the research studies to protect participants' rights) at (573) 882-3181.

You may ask more questions about the study at any time. For questions about the study or a research-related injury, contact Prof. Martin Katz at 573-882-8480 or via e-mail at katzm@health.missouri.edu.

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

Please complete the form below:

Name of blood donor: _____

Sex of blood donor: _____ Birth date of blood donor: _____

Date blood samples collected: _____

Has blood donor been diagnosed with LINCL? _____

If yes, was a DNA test part of the diagnosis? _____

If yes, can you provide the name of the gene with the mutation involved?

If the blood donor is not affected with NCL, is he/she related to a person with the disease? _____

If yes, relationship (father, mother, sister, brother, etc.) _____

Please list any dietary supplements and medications that the blood donor named above is taking:

Contact information of blood donor or guardian (optional, provide this information if you are willing to donate additional samples in the future or if you would like to be contacted about any findings of this study):

Signature

I confirm that the purpose of the research, the study procedures, the possible risks and discomforts as well as potential benefits that I may experience have been explained to me. Alternatives to my participation in the study also have been discussed. I have read this consent form and my questions have been answered. My signature below indicates my willingness to participate in this study.

Subject/Patient*

Date

Legal Guardian/Advocate/Witness (if required)**

Date

Additional Signature (if required) (identify relationship to subject)*** Date

*A minor's signature on this line indicates his/her assent to participate in this study. A minor's signature is not required if he/she is under 7 years old. Use the "Legal Guardian/Advocate/Witness" line for the parent's signature, and you may use the "Additional Signature" line for the second parent's signature, if required.

**The presence and signature of an impartial witness is required during the entire informed consent discussion if the patient or patient's legally authorized representative is unable to read.

***The "Additional Signature" line may be used for the second parent's signature, if required. This line may also be used for any other signature which is required as per federal, state, local, sponsor and/or any other entity requirements.

"If required" means that the signature line is signed only if it is required as per federal, state, local, sponsor and/or any other entity requirements.

SIGNATURE OF STUDY REPRESENTATIVE

I have explained the purpose of the research, the study procedures, identifying those that are investigational, the possible risks and discomforts as well as potential benefits and have answered questions regarding the study to the best of my ability.

Study Representative****

Date

***Study Representative is a person authorized to obtain consent. Per the policies of the University of Missouri Health Care, for any 'significant risk/treatment' study, the Study Representative must be a physician who is either the Principal or Co-Investigator. If the study is deemed either 'significant risk/non-treatment' or 'minimal risk,' the Study Representative may be a non-physician study investigator.

IF THE PATIENT IS INCOMPETENT TO GIVE CONSENT, COMPLETE THE FOLLOWING:

I, _____, hereby certify that I am _____
(Relationship to Patient)
of _____ and duly authorized to execute the foregoing.
(Name of Patient)

I consent to the blood sample donation as described in the attached consent form.

Legal Guardian/Patient Representative

Date

Study Representative*

Date

Witness (if required)**

Date

*Study Representative is a person authorized to obtain consent. Per the policies of the University of Missouri Health Care, for any 'significant risk/treatment' study, the study representative must be a physician who is either the Principal or Co-Investigator. If the study is deemed either 'significant risk/non-treatment' or 'minimal risk,' the study representative may be a non-physician study investigator.

**Regulations do not require the signature of a witness when the patient or patient's legally authorized representative is able to read and is capable of understanding the consent form document.

THE FOLLOWING REGULATION ONLY APPLIES WHEN AN ADULT PERSON, BECAUSE OF A MEDICAL CONDITION, IS TREATED AT A TEACHING HOSPITAL FOR A MEDICAL SCHOOL ACCREDITED BY THE AMERICAN OSTEOPATHIC ASSOCIATION OR THE AMERICAN MEDICAL ASSOCIATION AND SUCH PERSON IS INCAPABLE OF GIVING INFORMED CONSENT.

Persons authorized to consent when a patient is incapable of consenting to an experimental treatment, test or drug.

1. Legal guardian or
2. Attorney in fact (person appointed by durable power of attorney) or
3. A family member in the following order of priority:
 - a. Spouse unless the patient has no spouse, or is separated, or the spouse is physically or mentally incapable of giving consent, or the spouse's whereabouts is unknown or the spouse is overseas;
 - b. Adult child;

- c. Parent;
- d. Brother or sister;
- e. Relative by blood or marriage.

Such legal guardian, attorney in fact, or family member is not authorized to consent to treatment in contravention to such incapacitated person's expressed permission regarding such treatment.

If the patient is competent to consent but cannot write, do not use this proxy consent form. Please contact the IRB for directions in this situation.