Official Title: A Phase 1, Open-label Study to Validate Treatment-induced Biomarkers Following Sargramostim Treatment in Parkinson's Disease

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CONSENT FORM
Adult Consent Form

Title of this Research Study

A Phase 1, open-label study to validate treatment-induced biomarkers following sargramostim treatment in Parkinson's disease

Invitation and Summary

You are invited to be in this research study. Taking part in this research is voluntary. You do not have to take part. For the purposes of this document: "You" can refer to:

- Yourself
- The person for whom you are the Legally Authorized Representative (LAR)
- Your child under the age of 19.

"Organization" can refer to: University of Nebraska Medical Center (UNMC), Nebraska Medicine (NM), University of Nebraska at Omaha (UNO) or Children's Nebraska (CN).

Here is a summary of the purpose, methods, risks, benefits, and alternatives, to help you decide whether or not to take part in the research.

The goal of this study is to find disease links within the blood for early detection of Parkinson's disease (PD). You will have health, motor, and blood tests at three visits over the course of 48 weeks. You will also have a large blood collection during these visits. Possible risks include negative reactions to the study drug and/or during the blood collection process. Subjects are not expected to get any benefit from treatment. However, information from this study may benefit the patient population as a whole and may lead to a new therapy for PD. The alternative to being in the study is to not participate.

Why are you being asked to be in this research study?

You are being asked to take part in this study because you are between 35 and 85 years old and have Parkinson's disease (PD). You have had PD symptoms for at least 3 years, you can walk without help, and you can give your medications to yourself. Ten subjects will be enrolled and take part in the study and up to 15 subjects may be consented.

You must be willing to either self-administer study drug or have a caregiver help you.

If you are pregnant, nursing an infant, or plan to become pregnant during this study,

you may not be in this study.

If you have previously been given sargramostim or any form of granulocyte-macrophage colony-stimulating factor (GM-CSF), you cannot be in this study.

What is the reason for doing this research study?
The goal of this study is to test the safety of sargramostim in people with PD. Sargramostim (Leukine) is an FDA-approved drug, but it has not been approved for PD. It is currently used to treat patients with acute myelogenous leukemia, cancer patients undergoing chemotherapy, and patients who have had a bone marrow transplant.

This study will also collect large numbers of immune cells to track changes following treatment and to detect blood markers for disease diagnosis.

Some samples collected in the study will be given to external companies to complete some tests for this research study only. The results of this research study may help to make new therapies and/or assays to diagnose and treat PD.

What will be done during this research study?
You will be given information about the study and all your questions will be answered. If you would like to be in the study, you must sign this consent form before any study procedures begin.

This is a research study in which 10 PD subjects will participate and up to 15 subjects consented. The study will last a total of 48 weeks and includes 3 study visits. You will undergo a leukapheresis procedure to separate and collect white blood cells from red blood cells at each visit. During this process, you will be made comfortable in a bed. Then, you will have an IV placed in one arm for whole blood collection and a second IV placed in your other arm to return blood back into your bloodstream. You will need to remain still during the process, and you will not have use of your arms. This takes approximately 3 hours to complete.

Following this first blood collection, you will take study drug for a total of 48 weeks. This will be a daily injection for 5 days followed by a 2-day off period. You may give this injection yourself or it may be given by your caregiver. At each study visit, you will have a physical examination, motor examination, blood draw of up to 10 teaspoons, and leukapheresis. The blood draw will be used for 1) determining presence of GM-CSF antibodies; 2) screening for overall health (CBC/dif, metabolic panel); and 3) immune cell function and markers. After testing, samples will be

destroyed and will not be used for any commercial purposes.

Baseline Visit. At the initial screening visit, full informed consent will be obtained from all participants at the initial interview and enrollment, before any procedures have been performed. The initial interview will include a brief medical history and collection of demographic information. Blood will be drawn for a CBC and metabolic panel for health purposes only, and if necessary, for a pregnancy test for females of child-bearing age or to confirm menopause as well as determine hematocrit or hemoglobin levels and infectious disease profiles. Your veins will also be evaluated. Leukapheresis will be scheduled within the following week once blood work has been assessed and confirmed for continuation. A study investigator will also assess your physical health and perform motor evaluations.

Visits 1 - 3 : These will be your leukapheresis visits to isolate immune cells at baseline, 24 weeks, and 48 weeks of study drug. During the leukapheresis procedure, whole blood will be isolated for the same assessments as your baseline visit, and an investigator will perform an additional health and motor assessment. Following the initial leukapheresis procedure, you will begin study drug. The nursing staff will administer your first dose and train you on self-administration for future doses. You will also turn in adverse event logs and will also be given your drug supply for the next six months. Examples of adverse events would include any negative effect experienced during the course of drug administration such as pain, fatigue, headache, fever, site injection reactions, hives, vision problems, gut issues, sleep problems, falls etc.

At each visit, you will also be asked to complete a Parkinson's Disease Questionnaire 39 (PDQ39). This questionnaire is a 39-item self-reporting assessments that assess their general quality of life and well-being over the course of a month. You will also be assessed for competency and capacity to continue participating in the research. This assessment will be carried out by the study neurologist.

In between visits at 6 month intervals, the nurse coordinator will collect adverse event information via phone.

Adverse Event Appointments. If a study neurologist deems an additional visit is appropriate, such as with a subject who is experiencing an adverse event, the study will provide for such visits. If CBC/diff and comprehensive metabolic panel blood analyses are required at that time, the study will cover those also. The study will not cover other examinations or analyses that may be needed for an adverse event.

Additional Appointments. Additional visits may be necessary due to scheduling complications.

The sample(s) we collect will not be used for other research studies by us, or by any other investigator after this research is over.

The genetic tests will not include whole genome sequencing

Drug storage requirements.
Study drug will be provided as a powder with sterile water for mixing and insulin syringes for self-administration. The powder should be refrigerated, and once mixed, the liquid should be refrigerated and used within 24 hours. The powder form can be mixed as needed.

What are the possible risks of being in this research study?
Blood draw
Possible risks involved with the blood draw are minor pain and bleeding at the draw site, and a possible risk of infection. The usual sterile precautions will be observed to reduce the risk of infection. Rarely, a blood draw can cause fainting in some individuals.

Leukapheresis
The following are risks associated with leukapheresis which you may experience and which are similar to the risks associated with donation of a unit of whole blood: discomfort and/or bruising at the site of the needle sticks, nausea, vomiting, fainting or dizziness, blood loss, and infection.
In addition to these risks, possible complications of this leukapheresis procedure include:
1) side effects from citrate anticoagulant, the agent that prevents your blood from clotting: chilliness, tingling sensations, numbness, muscle cramping, and anxiety
2) allergic reactions to citrate, hydroxyethyl starch, or ethylene oxide, the agent used to sterilize the tubing and plastic bags in the blood cell separator: skin rash, hives, localized swelling, flushing, or difficulty breathing
3) complications from the infusion of hydroxyethyl starch: headache, fluid overload, ankle swelling, or high blood pressure Most of the starch will be excreted in your urine in the first 48 hours after the leukapheresis; however, traces of this starch may remain in your body for long periods of time. No harm has been shown to result from this. Platelets, red blood cells, and possibly some types of white blood cells may be depleted by frequent leukapheresis procedures. There are no harmful effects caused by the depletion of white cells from a single donation.

Overall, apheresis donations have acute reaction rates less than those seen with whole blood donation, although the frequency of reactions requiring hospitalization appears to be greater.

Sargramostim
The most common side effects from sargramostim are bone pain and injection site reactions. Headaches, fever, chills, muscle pain and weakness can also occur. Pain and fever are treated with acetaminophen, and injection site reactions are treated using ice compresses and changing injection sites.

Pregnancy Risks
It is possible that the medicines used in this study could injure a fetus or an unborn child if you or your partner becomes pregnant while taking them. You have already been told what is known about this possibility, and you are encouraged to ask further questions.
You may want to discuss this with others before you agree to take part in this study. If you wish, we will arrange for a doctor, nurse, or counselor who is not part of this study to discuss the potential risks and benefits with you and anyone else you want to have present. Because of the potential risks, you or your partner must not become pregnant while you are participating in this study. Women must have a negative pregnancy test before entering the study. If you are sexually active and can get pregnant, or can get your partner pregnant, you must use ONE appropriate method of birth control every time you have sex, or you must not have sex. You will need to continue to avoid pregnancy until you finish the research. By signing this and being in the study, you are agreeing to not get your partner pregnant while you are on the study. Should you become pregnant while you are on this study, you should immediately notify the study personnel. The investigator will assist you in finding appropriate medical care. The investigator also may ask to be allowed to continue getting information about her pregnancy. You can refuse to provide this information.

Other Risks
It is possible that other rare side effects could occur which are not described in this consent form. It is also possible that you could have a side effect that has not occurred before. It is also possible your Parkinson's disease may become worse while participating in this study.

Laboratory Tests
There is no known risk to you from the laboratory research which will be performed using your leukocytes or blood.

What are the possible benefits to you?
You are not expected to get any benefit from being in this research study.

What are the possible benefits to other people?
This study may lead to new ways to diagnose and treat PD. This study may also show whether sargramostim is safe and tolerable for people with PD and whether it can change their immune cells, which may or may not lead to future treatments.

What are the alternatives to being in this research study?
Instead of being in this research study, you can choose not to take part.

What will being in this research study cost you?
There is no cost to you to be in this research study.

Will you be paid for being in this research study?
You will be paid at the rate of $15/hr for your participation in this study. If you are unable to complete the blood collection procedure, the amount will be prorated according to time spent. In the event that any commercial products are developed from your tissue/blood, UNMC/Nebraska Medicine has no plans to share with you any proprietary interests in the products or revenue generated from such commercialization. All donated tissue/blood is the property of the University of Nebraska Medical Center (UNMC).

For your time and travel for this study, you will receive compensation in the form of a University of Nebraska check for each study visit completed in the following amounts:

Visit 1a Enrollment (1 hr): $15
Visit 1b Leukapheresis (3 hr): $45
Visit 2 Leukapheresis (3 hr): $45
Visit 3 Leukapheresis (3 hr): $45

Adverse Event Appointments (2 hrs): $30

If you need assistance for travel expenses, please discuss this with your study coordinator. You will be requested to provide receipts for reimbursement for travel fares such as taxi fare, bus tickets, etc, for each visit. Air fare will not be covered.

To receive payment, you must provide your social security number, name, and address in order to comply with Internal Revenue Service (IRS) reporting requirements. When payment is reported to the IRS, we will not say what the

payment is for, only that you have been paid. If you do not wish to provide this information, you can still participate in the study; however, you will not be paid.

Who is paying for this research?
This research is being paid for by Nebraska Neuroscience Alliance: Community Based Consortium of Private Donators. The investigator receives the study drug and additional funding from Partner's Therapeutics to conduct this study.

Dr. Gendelman and Dr. Mosley are co-inventors of an invention that utilizes sargramostim/GM-CSF as part of a vaccine for PD. This study may help validate their vaccine study. Results of this study may be used for commercial purposes.

What should you do if you are injured or have a medical problem during this research study?
Your health and safety is our main concern. If you are injured or have a medical problem because of this study call someone listed at the end of this consent form. You can get emergency medical treatment at Nebraska Medicine. You can also go to your doctor, the nearest emergency room or call 9-1-1.

We have no plans to pay for your treatment or give you any other money or compensation. Your insurance may pay. If they do not you will have to pay.

Signing this does not mean you have given up any of your legal rights.

How will information about you be protected?
In the course of this research we may collect information about you. This can be things that could be used to find out who you are (like your name, phone number, birthdate, address). We call this "identifiable private information". We will keep this information as confidential as possible. Additionally, the information will not be used for other research by us, or by any other researcher.

Who can see information about you?
We also will get medical information about you (like medical record number, medical history, or the results of physical exams, blood tests, x-rays or other medical or research procedures). We call this "protected health information" or PHI. PHI is protected by a law called the HIPAA Privacy Rule. We will collect the smallest amount of PHI that we can. We will keep your PHI as confidential as possible.

By signing this consent form, you are letting us (the researchers listed on this consent form and other people involved in this research at the Organization) have

access to your PHI. Your PHI will be used only for the purposes described in the section "What is the reason for doing this research study?"

You can change your mind and tell us to stop collecting your PHI for use in this research at any time by writing to the principal investigator. We can still use the PHI we have already collected. If you tell us to stop collecting your PHI, you will have to stop being in this research.

We may share your PHI with other groups listed below:

- The UNMC Institutional Review Board (IRB)
- Institutional officials designated by the UNMC IRB
- The HHS Office for Human Research Protections (OHRP)
- The Food and Drug Administration (FDA)
- Partner Therapeutics, which sponsors this research and may pay the Organization to do this research
- The Data and Safety Monitoring Board (DSMB)

The Privacy Rule may not apply to all of the above groups. Once disclosed outside of UNMC federal privacy laws may no longer protect your PHI. Ask the investigator (or contact the Office of Regulatory Affairs at IRBORA@unmc.edu) if you have questions.

You are letting us use and share your research data for as long as the research is going on.

How will results of the research be made available to you during and after the study is finished?

Information obtained in the course of the research that will not be shared with you is the characterization of your blood cells and any genetic information obtained from this. By signing this authorization, you are temporarily giving up your right to see this research-related information while the research is going on. You will be able to see this information if you wish after the research is completed.

In most cases, the results of the research can be made available to you when the study is completed, and all the results are analyzed by the investigator or the sponsor of the research. The information from this study may be published in scientific journals or presented at scientific meetings, but your identity will be kept strictly confidential.

If you want the results of the study, contact the Principal Investigator at the phone

number given at the end of this form or by writing to the Principal Investigator at the following address:

Howard E. Gendelman, M.D.
Department of Pharmacology and Experimental Neuroscience
University of Nebraska Medical Center (DRC 3005)
985800 Nebraska Medical Center
Omaha, NE 68198-5800

A description of this clinical trial will be available onwww.clinicaltrials.gov, as required by U.S. law. This website will not include information that can identify you. At most, the website will include a summary of the results. You can search this website at any time.

What will happen if you decide not to be in this research study?
You can decide not to be in this research study. Deciding not to be in this research will not affect your medical care or your relationship with the investigator or the organization. Your doctor will still take care of you and you will not lose any benefits to which you are entitled.

What will happen if you decide to stop participating once you start?
You can stop being in this research (withdraw) at any time. If you decide to stop being in the research, please let us know. If you stop being in the research study it will not affect your care or your relationship with the investigator or the organization. You will not lose any benefits to which you are entitled.

For your safety, please talk to the research team before you stop taking any research medicine or treatments. They will tell you how to do it safely. They may ask you if you will have some extra tests. You do NOT have to agree to do these tests.

You may be taken off the study if you do not follow instructions of the investigator or the research team.

Any research data we have already collected can still be used in the research.

Will you be given any important information during the study?
You will be informed promptly if the research team gets any new information during this research study that may affect whether you would want to continue being in the study.

What should you do if you have any questions about the study?
We gave you a copy of *"What Do I Need to Know Before Being in a Research Study?"* If you ever have any questions about this study, call the Principal Investigator or anyone else listed on this consent form.

What are your rights as a research participant?
You have rights as a research subject. These rights have been explained in this consent form and in The Rights of Research Subjects that you have been given. If you have any questions concerning your rights, or want to discuss problems, concerns, obtain information or offer input, or make a complaint about the research, you can contact any of the following:

- The investigator or other study personnel
- Institutional Review Board (IRB)
    - Telephone: (402) 559-6463
    - Email: IRBORA@unmc.edu
    - Mail: UNMC Institutional Review Board, 987830 Nebraska Medical Center, Omaha, NE 68198-7830
- Research Subject Advocate
    - Telephone: (402) 559-6941
    - Email: unmcrsa@unmc.edu

Documentation of informed consent
You are deciding whether to be in this research study. Signing means that:

- You have read and understood this consent form.
- You have had the consent form explained to you.
- You have been given a copy of The Rights of Research Subjects
- You have had your questions answered.
- You have decided to be in the research study.
- You have been told you can talk to one of the researchers listed below on this consent form if you have any questions during the study.
- You will be given a signed and dated copy of this consent form to keep.

Signature of Subject_____ Date_____

My signature certifies that all the elements of informed consent described on this consent form have been explained fully to the subject. In my judgment, the subject possesses the legal capacity to give informed consent to participate in this research and is voluntarily and knowingly giving informed consent to participate.

Signature of Person Obtaining Consent_____
Date_____

Authorized Study Personnel
Principal
Gendelman, Howard
phone: 402-559-5478
alt #: 402-559-8920
degree: MD

Secondary
Mosley, R Lee
phone: 402-559-2510
alt #: 402-559-2510
degree: PhD

* Santamaria, Pamela
alt #: 531-999-2670
degree: MD

Lead Coordinator
Olson-Johnson, Katherine
alt #: 402-559-2547
degree: PhD

Other Coordinator
Obaro, Helen
alt #: 402-559-0964
degree: RN, BSN

Ostlund, Katie
phone: 402-559-9581
alt #: 402-559-7685
degree: MS

What Do I Need To Know Before Being In A Research Study?

You have been invited to be in a **research study**. Research studies are also called "clinical trials" or "protocols." **Research** is an organized plan designed to get new knowledge about a disease or the normal function of the body. The people who are in the research are called **research subjects**. The **investigator** is the person who is running the research study. You will get information from the investigator and the research team, and then you will be asked to give your **consent** to be in the research.

This sheet will help you think of questions to ask the investigator or his/her staff. You should know all these answers before you decide about being in the research.

What is the **purpose** of the research? Why is the investigator doing the research?

What are the **risks** of the research? What bad things could happen?

What are the possible **benefits** of the research? How might this help me?

How is this research different than the care or treatment I would get if I wasn't in the research? Are there other treatments I could get?

Does **everyone** in this research study get the same treatment?

Will being in the research **cost** me anything extra?

Do I have to be in this research study? Will the doctor still take care of me if I say **no**?

Can I **stop** being in the research once I've started? How?

Who will look at my **records**?

How do I reach the investigator if I have more **questions**?

Who do I call if I have questions about being a **research subject**?

Make sure all your questions are answered before you decide whether or not to be in this research.

Academic Research & Services Building 3000 / 987830 Nebraska Medical Center / Omaha NE 68198-7830
402-559-6463 / FAX 402-559-3300 / Email: irbora@unmc.edu / http://www.unmc.edu/irb

THE RIGHTS OF RESEARCH SUBJECTS
AS A RESEARCH SUBJECT YOU HAVE THE RIGHT ...

... to be told everything you need to know about the research before you are asked to decide whether or not to take part in the research study. The research will be explained to you in a way that assures you understand enough to decide whether or not to take part.

... to freely decide whether or not to take part in the research.

... to decide not to be in the research, or to stop participating in the research at any time. This will not affect your medical care or your relationship with the investigator or the Nebraska Medical Center. Your doctor will still take care of you.

... to ask questions about the research at any time. The investigator will answer your questions honestly and completely.

... to know that your safety and welfare will always come first. The investigator will display the highest possible degree of skill and care throughout this research. Any risks or discomforts will be minimized as much as possible.

... to privacy and confidentiality. The investigator will treat information about you carefully, and will respect your privacy.

... to keep all the legal rights you have now. You are not giving up any of your legal rights by taking part in this research study.

... to be treated with dignity and respect at all times

The Institutional Review Board is responsible for assuring that your rights and welfare are protected. If you have any questions about your rights, contact the Institutional Review Board at (402) 559-6463.