PRINCIPAL INVESTIGATOR: Staci Martin, Ph.D.

STUDY TITLE: Pilot Study of Medication Adherence in Children, Adolescents and Adults with Neurofibromatosis Type 1 (NF1) on Clinical Treatment Trials

STUDY SITE: National Institutes of Health Clinical Center

Cohort: Standard

Consent Version: 11/12/2020

WHO DO YOU CONTACT ABOUT THIS STUDY?

Staci Martin, Ph.D.
Phone: (240) 760-6203
Email: martins@mail.nih.gov

Andrea Gross, M.D.
Telephone: 240-858-3853
Email: andrea.gross@nih.gov

This consent form describes a research study and is designed to help you decide if you would like to be a part of the research study.

You are being asked to take part in a research study at the National Institutes of Health (NIH). Members of the study team will talk with you about the information described in this document. Some people have personal, religious, or ethical beliefs that may limit the kinds of medical or research treatments they would want to receive (such as blood transfusions). Take the time needed to ask any questions and discuss this study with NIH staff, and with your family, friends, and personal health care providers. Taking part in research at the NIH is your choice.

If the individual being enrolled is a minor, then the term "you" refers to "you and/or your child" throughout the remainder of this document.

IT IS YOUR CHOICE TO TAKE PART IN THE STUDY

You may choose not to take part in this study for any reason. If you join this study, you may change your mind and stop participating in the study at any time and for any reason. In either case, you will not lose any benefits to which you are otherwise entitled. However, to be seen at the NIH, you must be taking part in a study or are being considered for a study. If you do choose to leave the study, please inform your study team to ensure a safe withdrawal from the research.

WHY IS THIS STUDY BEING DONE?

The primary purpose of this study is to assess how often people with Neurofibromatosis type 1 (NF1) take medications that have been prescribed to them for treatment of plexiform neurofibroma(s). Treatment for plexiform neurofibromas with daily medication may require taking medicine for a long period of time (months or years), and we believe that it will be important for patients to take the medication regularly for it to be effective.

In this study, we will use a set of questionnaires to gather background information including information about any recent major life events as well as to evaluate what might interfere with people with NF1 taking a medication regularly.

WHY ARE YOU BEING ASKED TO TAKE PART IN THIS STUDY?

You have been diagnosed with NF1 and a plexiform neurofibroma and have been enrolled on a clinical trial to treat the plexiform neurofibroma with a medication that you will take at least once a day.

HOW MANY PEOPLE WILL TAKE PART IN THIS STUDY?

Up to 30 people will take part in this study. This is includes 12 to 15 people with NF1, as well as parents and guardians of some of these patients.

DESCRIPTION OF RESEARCH STUDY

WHAT WILL HAPPEN IF YOU TAKE PART IN THIS RESEARCH STUDY?

Before you begin the study

A treatment team member referred you to this study. To make sure you meet the requirements for taking part in this study we will talk to your medical doctor. We will also make sure you have access to the internet and equipment that will allow you to do the protocol activities.

During the study

Once you sign this consent document, you will be asked to complete five questionnaires. The questionnaires should take about 20 minutes to complete.

The baseline questionnaires will collect information about the following areas:

1. Demographic questionnaire: Basic information about demographic and educational history;
2. Recent Life Events: Checklist of major changes in your personal or professional life in the last several months;
3. Pain interference: How much pain interferes with your daily activities;
4. Cognitive Function: Includes how you are able to focus and pay attention to daily tasks
5. Emotional Distress/Depression: Evaluates for any signs of emotional distress or depression

During the course of the study you will be asked to mark down each time you take a dose of the NF1 study medication on a medication diary form we will provide to you. In addition, you will be asked to use a special bottle cap that includes a microchip. This microchip will track the dates and times you open your bill bottle. We may also ask you to complete a short questionnaire and/or participate in a brief interview with our team to discuss what type of intervention might help you remember to take your medication regularly. The interview will be conducted over the phone or on videoconference and should not take longer than 30 minutes.

RISKS OR DISCOMFORTS OF PARTICIPATION

What side effects or risks can I expect from being in this study?

The time commitment for the completion of questionnaires is the primary burden of participation. In addition, some individuals experience anxiety or stress related to talking about remembering to take their medication.

POTENTIAL BENEFITS OF PARTICIPATION

Are there benefits to taking part in this study?

We do not know if you will receive direct benefit for participation in this study, however you will help us learn more about how to help make sure that other patients with NF1 get the most benefit from their prescribed medicines.

ALTERNATIVE APPROACHES OR TREATMENTS

WHAT OTHER CHOICES DO I HAVE IF I DO NOT TAKE PART IN THIS STUDY?

You have the option to not participate in this study. You may still participate in any other support therapies offered by NIH. If you choose not to participate, it will not affect your medical care or participation in any other research study at NIH.

STOPPING PARTICIPATION

The study doctor may decide to stop your participation for the following reasons:

- you have completed the study follow-up period
- you stop participating in your treatment study
- if he/she believes that it is in your best interest

In this case, you will be informed of the reason therapy is being stopped.

You can stop taking part in the study at any time. However, if you decide to stop taking part in the study, we would like you to talk to the study investigator first.

COMPENSATION, REIMBURSEMENT, AND PAYMENT

Will you receive compensation for participation in the study?

Some NIH Clinical Center studies offer compensation for participation in research. The amount of compensation, if any, is guided by NIH policies and guidelines.

On this study you will receive:

- $25 for completion of surveys
- $15 for participating in the phone interviews

We will compensate you, via direct deposit or check, to thank you for your time in filling out the questionnaires, medication diaries, and participating in interviews.

If you are unable to finish the study, you will receive compensation for the parts you completed.

With few exceptions, study compensation is considered taxable income that is reportable to the Internal Revenue Service (IRS). A "Form 1099-Other Income" will be sent to you if your total

payments for research participation are $600 or more in a calendar year. If you have unpaid debt to the federal government, please be aware that some or all of your compensation may be automatically reduced to repay that debt on your behalf.

Will you receive reimbursement or direct payment by NIH as part of your participation?

Some NIH Clinical Center studies offer reimbursement or payment for travel, lodging or meals while participating in the research. The amount, if any, is guided by NIH policies and guidelines.

This study does not offer reimbursement for, or payment of, travel, lodging or meals.

Will taking part in this research study cost you anything?

NIH does not bill health insurance companies or participants for any research or related clinical care that you receive at the NIH Clinical Center.

- If some tests and procedures are performed outside the NIH Clinical Center, you may have to pay for these costs if they are not covered by your insurance company.
- Medicines that are not part of the study treatment will not be provided or paid for by the NIH Clinical Center.
- Once you have completed taking part in the study, medical care will no longer be provided by the NIH Clinical Center.

CLINICAL TRIAL REGISTRATION AND RESULTS REPORTING

A description of this clinical trial will be available on http://www.ClinicalTrials.gov, as required by U.S. Law. This Web site will not include information that can identify you. At most, the Web site will include a summary of the results. You can search this Web site at any time.

CONFIDENTIALITY PROTECTIONS PROVIDED IN THIS STUDY

Will your medical information be kept private?

We will do our best to make sure that the personal information in your medical record will be kept private. However, we cannot guarantee total privacy. Organizations that may look at and/or copy your medical records for research, quality assurance, and data analysis include:

- The NIH and other government agencies, like the Food and Drug Administration (FDA), which are involved in keeping research safe for people.
- National Institutes of Health Intramural Institutional Review Board
- The Principal Investigator of the clinical trial you are enrolled on for the treatment of your NF1.

When results of an NIH research study are reported in medical journals or at scientific meetings, the people who take part are not named and identified. In most cases, the NIH will not release any information about your research involvement without your written permission. However, if you sign a release of information form, for example, for an insurance company, the NIH will give the insurance company information from your medical record. This information might affect (either favorably or unfavorably) the willingness of the insurance company to sell you insurance.

If we share your specimens or data with other researchers, in most circumstances we will remove your identifiers before sharing your specimens or data. You should be aware that there is a slight possibility that someone could figure out the information is about you.

Further, the information collected for this study is protected by NIH under a Certificate of Confidentiality and the Privacy Act.

Certificate of Confidentiality

To help us protect your privacy, the NIH Intramural Program has received a Certificate of Confidentiality (Certificate). With this certificate, researchers may not release or use data or information about you except in certain circumstances.

NIH researchers must not share information that may identify you in any federal, state, or local civil, criminal, administrative, legislative, or other proceedings, for example, if requested by a court.

The Certificate does not protect your information when it:

1. is disclosed to people connected with the research, for example, information may be used for auditing or program evaluation internally by the NIH; or
2. is required to be disclosed by Federal, State, or local laws, for example, when information must be disclosed to meet the legal requirements of the federal Food and Drug Administration (FDA);
3. is for other research;
4. is disclosed with your consent.

The Certificate does not prevent you from voluntarily releasing information about yourself or your involvement in this research.

The Certificate will not be used to prevent disclosure to state or local authorities of harm to self or others including, for example, child abuse and neglect, and by signing below you consent to those disclosures. Other permissions for release may be made by signing NIH forms, such as the Notice and Acknowledgement of Information Practices consent.

Privacy Act

The Federal Privacy Act generally protects the confidentiality of your NIH medical records we collect under the authority of the Public Health Service Act. In some cases, the Privacy Act protections differ from the Certificate of Confidentiality. For example, sometimes the Privacy Act allows release of information from your medical record without your permission, for example, if it is requested by Congress. Information may also be released for certain research purposes with due consideration and protection, to those engaged by the agency for research purposes, to certain federal and state agencies, for HIV partner notification, for infectious disease or abuse or neglect reporting, to tumor registries, for quality assessment and medical audits, or when the NIH is involved in a lawsuit. However, NIH will only release information from your medical record if it is permitted by both the Certificate of Confidentiality and the Privacy Act.

USE OF DATA FOR FUTURE RESEARCH

To advance science, it is helpful for researchers to share information they get from their data. They do this by putting it into one or more scientific databases, where it is stored along with information

from other studies. A researcher who wants to study the information must apply to the database and be approved. Researchers use data stored in scientific databases to advance science and learn about health and disease.

We plan to keep some of your data that we collect and use them for future research and share them with other researchers. We will not contact you to ask about each of these future uses. These data will be stripped of identifiers such as name, address or account number, so that they may be used for future research on any topic and shared broadly for research purposes. Your data will be used for research purposes only and will not benefit you. It is also possible that the stored data may never be used. Results of research done on your data will not be available to you or your doctor. It might help people who have cancer and other diseases in the future.

If you do not want your stored data used for future research, please contact us in writing and let us know that you do not want us to use your data. Then any data that have not already been used or shared will be destroyed and will not be used for future research. However, it may not be possible to withdraw or delete materials or data once they have been shared with other researchers.

POLICY REGARDING RESEARCH-RELATED INJURIES

The NIH Clinical Center will provide short-term medical care for any injury resulting from your participation in research here. In general, no long-term medical care or financial compensation for research-related injuries will be provided by the NIH, the NIH Clinical Center, or the Federal Government. However, you have the right to pursue legal remedy if you believe that your injury justifies such action.

PROBLEMS OR QUESTIONS

If you have any problems or questions about this study, or about your rights as a research participant, or about any research-related injury, contact the Principal Investigator, Staci Martin, Ph.D. martins@mail.nih.gov, 240-760-6025. *Other researchers you may call are: Andrea Gross, M.D. at 240-858-3853.* You may also call the NIH Clinical Center Patient Representative at 301-496-2626, or the NIH Office of IRB Operations at 301-402-3713, if you have a research-related complaint or concern.

CONSENT DOCUMENT

Please keep a copy of this document in case you want to read it again.

Adult Research Participant: I have read the explanation about this study and have been given the opportunity to discuss it and to ask questions. I consent to participate in this study.

Signature of Research Participant	Print Name of Research Participant	Date

Parent/Guardian of a Minor Participant: I have read the explanation about this study and have been given the opportunity to discuss it and to ask questions. I give permission for my child to take part in this study.

Signature of Parent/Guardian	Print Name of Parent/Guardian	Date
Signature of Parent/Guardian	Print Name of Parent/Guardian	Date

Investigator:

Signature of Investigator	Print Name of Investigator	Date