Informed Consent Form

TITLE: iPS Cell Response to CFTR Modulators: Study of Trikafta in CF Patients Carrying Partial Function Mutations or N1303K CFTR

NCT NUMBER: NCT03506061

IRB APPROVAL DATE: September 7, 2022

You Are Being Asked to Be in a Research Study

What Is a Research Study?

The main purpose of research studies is to gain knowledge. This knowledge may be used to help others. Research studies are not intended to benefit you directly, though some might.

Do I Have to Do This?

No. Being in this study is entirely your choice. If you decide to join this study, you can change your mind later on and withdraw from the research study.

Taking part in a study is separate from medical care. The decision to join or not join the research study will not affect your status as a patient.

What Is This Document?

This form is an informed consent document. It will describe the study risks, procedures, and any costs to you.

This form is also a HIPAA Authorization document. It will describe how your health information will be used and by whom.

Signing this form indicates you are willing to take part in the study and allow your health information to be used.

What Should I Do Next?

1. Read this form, or have it read to you.
2. Make sure the study Doctor or study staff explains the study to you.
3. Ask questions (e.g., time commitment, unfamiliar words, specific procedures, etc.)
4. If there will be medical treatment, know which parts are research and which are standard care.
5. Take time to consider this, and talk about it with your family and friends.

Emory University
Consent to be a Research Subject / HIPAA Authorization

Title: iPS cell response to CFTR modulators: Study of Trikafta™ in CF patients carrying partial function mutations or N1303K CFTR

Principal Investigator: Eric Sorscher, MD, Department of Pediatrics; Rachel Linnemann, MD, Department of Pediatrics

Study-Supporter: National Institute of Health and Cystic Fibrosis Foundation

If you are the legal guardian of a child who is being asked to participate, the term "you" used in this consent refers to your child

Introduction

You are being asked to be in a medical research study. This form is designed to tell you everything you need to think about before you decide if you want to be a part of the study. **It is entirely your choice. If you decide to take part, you can change your mind later on and withdraw from the research study.** The decision to join or not join the research study will not cause you to lose any medical benefits. If you decide not to take part in this study, your Doctor will continue to treat you.

Before making your decision:

- Please carefully read this form or have it read to you
- Please listen to the study Doctor or study staff as they explain the study to you
- Please ask questions about anything that is not clear

You can take a copy of this consent form, to keep. Feel free to take your time thinking about whether you would like to participate. You may wish to discuss your decision with family or friends. Do not sign this consent form unless you have had a chance to ask questions and get answers that make sense to you. By signing this form you will not give up any legal rights.

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.

What is the purpose of this study?

Cystic Fibrosis (CF) is a disease caused by mutations (errors) in the gene for a protein present on the cell surface (called CFTR "cystic fibrosis transmembrane conductance regulator"). These defects prevent the correct movement of chloride in your cells. The purpose of this study is to learn whether cells taken from a small piece of skin and/or small sample of blood can be used to predict benefits in lung function from the cystic fibrosis drug Trikafta™. Trikafta™ is an oral drug that overcomes certain CFTR mutations. Trikafta™ is approved for patients with CF but is not approved for patients with your specific mutation.

This is a pilot study since it is the first time cells taken from the skin or blood have been tested as a way to predict whether patients with a certain form of CF will respond to a drug therapy such as Trikafta™. We are testing this drug on your mutation to learn if and how it helps CF.

What will I be asked to do?

If you qualify for the study, you will receive orally administered Trikafta™ (elexacaftor 100 mg/tezacaftor 50 mg/ivacaftor 75 mg (2 pills once daily in the morning) and ivacaftor (150 mg) once daily in the evening. You will take the medicine for four weeks.

After the 4 week period, you will return to our office 4 weeks later for a follow up visit. Trikafta™ is a new oral drug approved by the FDA for the treatment of CF due to its ability to correct a basic defect in some forms of cystic fibrosis. Researchers have found evidence that there may be a chance this drug could help people with your mutation. The drug should be taken with a regular meal of your choice.

You will need to take part in 5 office visits and 1 telephone visit. If you are entered and complete the entire study, you will be in the study for about 3 months. We hope to enroll around 11 participants who are ages 12 years old and older at Emory with evidence of partial CFTR function and an additional 10 participants with the N1303K mutation who are 12 years and older at Emory. The following test and procedures will be done at each study visit. All procedures will be explained later in the consent and again in detail by the study Doctor.

People who enter into the study will take the drug, Trikafta™. Each procedure is explained in detail (later in this form) following the visit description.

Screening (28 Day Screening Period): this visit will take approximately 4-6 hours. We will discuss the study with you and answer any questions. After signing the consent form the following procedures will be performed. During this visit about 1-2 tablespoons of blood (depending on weight) will be collected from a vein in your arm.

- Medical History including your past lung health, previous diagnosis, etc
- Vital Signs including oxygen (O_2) Saturation
- Medication Review
- Physical Exam, the study Doctor will listen to your heart, lungs, etc
- CFTR Genotyping (to confirm your CFTR mutation)
- Safety laboratory test/Urinalysis
- Serum Pregnancy Test for Females
- Spirometry
- Sweat Chloride Collection
- EKG
- Eye exam (if not done within 3 months before screening for those ages 12-17 at the time of screening)
- Questionnaire

Day 1: this visit will take approximately 5-6 hours. During the visit about 1-2 tablespoons of blood (depending on weight) will be drawn. The following procedures will be performed.

- Vital Signs including O_2 Saturation
- Adverse Event Review
- Interval Medical history
- Medication Review
- Physical Exam, the study Doctor will listen to your heart, lungs, etc
- Spirometry
- Sweat Chloride Collection
- Safety laboratory test/Urinalysis
- Urine Pregnancy Test for females

- 3 mm Punch Biopsy of skin and/or blood sample (can occur any time between Screening and Day 1). If you are a person with a partial CFTR function, you will need to provide a skin biopsy. If you are a person with N1303K, you will need to provide a blood sample and will have the option to provide a skin biopsy.
- Questionnaire
- Drug Dispensing
- Drug Administration

Day 7 (+/- 2 days) this will be a telephone contact and will take approximately 30 minutes. The following topics will be discussed.

- Adverse Event Review
- Interval Medical history
- Medication Review

Day 14 (+/- 2 days): this visit will take approximately 5-6 hours. The following procedures will be performed. During this visit about 1-2 tablespoons of blood (depending on weight) will be drawn.

- Vital Signs including O_2 Saturation
- Adverse Event Review
- Interval Medical history
- Medication Review
- Physical Exam, the study Doctor will listen to your heart, lungs, etc
- Spirometry
- Safety laboratory test/Urinalysis
- EKG
- Sweat Chloride Collection
- Questionnaire
- Drug Dispensing
- Drug Collection
- Drug Administration

Day 28 (+1 / - 2 days): this visit will take approximately 5-6 hours. The following procedures will be performed. During this visit about 1-2 tablespoons of blood (depending on weight) will be drawn.

- Vital Signs including O_2 Saturation
- Adverse Event Review
- Interval Medical history
- Medication Review
- Physical Exam, the study Doctor will listen to your heart, lungs, etc
- Spirometry
- Safety laboratory test/Urinalysis
- Sweat Chloride Collection
- Questionnaire
- Drug Dispensing
- Drug Collection
- Drug Administration

Day 56 (+/- 2 days): this visit will take approximately 5-6 hours. The following procedures will be performed. During this visit about 1-2 tablespoons of blood will be drawn.

- Vital Signs including O_2 Saturation
- Adverse Event Review
- Interval Medical history
- Medication Review
- Physical Exam, the study Doctor will listen to your heart, lungs, etc
- Spirometry
- Urine Pregnancy Test for females
- Sweat Chloride Collection
- 3 mm Punch Biopsy of skin and/or blood sample if necessary to obtain additional cells (may occur within a few days before or after day 56 based on dermatology clinic scheduling)
- Safety laboratory test/Urinalysis
- Questionnaire

Questionnaires:
You will be asked questions about the history of your health and cystic fibrosis.

Physical Exam:
This exam will include the following: blood pressure, height, weight, heart rate, temperature, respiration rate, general appearance, general impression of your chest, heart, head, eyes, ears, nose, throat, neck, abdomen, extremities, skin, neurological and any other notes made by the health care provider.

Blood Draw:
About 1-2 tablespoons of blood will be taken from your arm for safety and other parameters at each visit.

Pregnancy Test:
Females who are pregnant or nursing a child cannot be in this study. If there is a physical possibility of being or getting pregnant, a serum pregnancy test followed by urine pregnancy tests may be given.

Spirometry:
This is a test that measures how well you breathe. You will wear a nose clip and breathe out forcefully into a machine called a spirometer. This machine measures how much air you blow out and how fast it comes out. If possible, do not take bronchodilators (medication that opens up the airways) the morning of each visit. (Depending on which bronchodilators you use, your Study Doctor may tell you not to take them for 12 – 24 hours before each visit.)

Sweat Chloride Test:
In the sweat test, a place on your arm is stimulated with electrodes to produce sweat. The sweat is caught in a collector disc placed on the skin. We will measure chemicals, such as salt, in your sweat. To collect the sweat, 2 probes will be attached to the skin on your arm for 4-5 minutes. A gel-like medicine called pilocarpine is put on the probes and causes the sweat glands to produce more sweat. The probes stay on for about 4-5 minutes, then are removed and replaced by a disc to collect sweat for about 30 minutes. The entire procedure will then be repeated on the other arm.

Punch Biopsy
A sample of your skin will be taken (called a punch biopsy) after numbing the area with a local anesthetic. The circular area of skin taken will be similar in size to a small round green pea. The biopsy will be about 3 mm in diameter and about 1-2 mm thick. A punch biopsy will be collected once during the study, and may need to be repeated on the last day of the study if additional biopsy material is needed. The biopsy site will then be sewn up, usually with one or two stitches.

EKG
ECGs will be performed to check the electrical activity of your heart. This test is painless and takes about 10 minutes.

Eye Exam:
If you are 12-17 years of age you will have one eye examination unless you have had an eye exam in the last three months. The examination will take place at the time of the screening visit.

How will my medicine be provided?
The medicine that you will take will be dispensed by the pharmacy and delivered to the principal investigator or study team member. The principal investigator or health care providers on his/her research team will provide the medicine to you. If you have questions about the medicine, you should ask the principal investigator or study nurse. You may also call the pharmacy if you have questions about the medicine. The number for the pharmacy is included on your medicine package.

Who owns my study information and samples?
If you join this study, you will be donating your samples and study information. You will not receive any compensation if your samples or information are used to make a new product. If you withdraw from the study, data and samples that were already collected may be still be used for this study.

What are the possible risks and discomforts?
There may be side effects from the study drug or procedures that are not known at this time. Examples of adverse reactions known in subjects aged 12 years and older are listed below.

The most common (occurring 5% or more) risks and discomforts expected in this study are:

Headache	17%
Upper respiratory tract infection	16%
Abdominal pain	14%
Diarrhea	13%
Rash	10%
ALT increased (a liver test)	10%
Nasal congestion	9%
Blood CPK increased (a muscle test)	9%
Rhinorrhea (runny nose)	8%
Rhinitis (nose irritation)	7%
AST increased (a liver test)	9%
Influenza	7%
Sinusitis	5%
Blood bilirubin increased (a liver test)	5%

High liver enzymes (called ALT or AST) in the blood have also been observed in some subjects. The very high levels of these tests could lead to stopping of Study Drug, and these abnormal blood tests may get better after Study Drug is stopped. In some severe cases, high liver enzymes may be shown as a sign of liver injury, and can become permanent and even be life-threatening.

Eye examinations performed in studies involving subjects less than 12 years of age receiving long term ivacaftor (a component of the Trikafta™ treatment), have identified several subjects with cataracts (cloudiness of the lens of the eye). Ivacaftor may be associated with cataracts.

Possible Risks Based on Animal Studies
In a study in which ivacaftor (a drug found in Trikafta™) was given to newborn rats, cataracts (cloudiness of the lens of the eye) were seen. No cataracts were seen in studies of older animals (rats and dogs) dosed with ivacaftor for longer periods of time. The importance of this finding in humans is unknown.

Drug Interaction Risks (medicines working with or against each other):
Almost all medicines can cause side effects. Many are mild, but some can sometimes become life-threatening, particularly if they are not treated. The combination of the Study Drug and any other medications, dietary supplements, natural remedies, and vitamins could be harmful to you. It is very important that you tell your study Doctor about every medicine, dietary supplement, natural remedy, and vitamin (or change) while you are in the Study. There are certain herbal medications, like St. John's Wort, and certain fruits and fruit juices, such as grapefruit or grapefruit juice, that you must not take during Study. Your study Doctor will review these with you.

Risks of Discontinuing Study Medication:
Once the four-week treatment period is complete, you will no longer receive Trikafta™. There is a chance that your CF will improve when taking Trikafta™. However, your CF symptoms may appear to worsen after you stop taking Trikafta™ as you return to a respiratory baseline.

If you are a woman: to protect against possible side effects of the study drug, women who are pregnant or nursing a child may not take part in this study. If you become pregnant, there may be risks to you, the embryo, or fetus. These risks are not yet known. If you are a woman of childbearing ability, you and the study Doctor must agree on a method of birth control to use throughout the study. If you think that you have gotten pregnant during the study, you must tell the study Doctor immediately. Pregnant women will be taken out of the study.

If you are a man: the effect of the study drug on sperm is not known. To protect against possible side effects, if you are a man and your female partner becomes pregnant during the Study, you should notify the study Doctor right away. If you or your female partner becomes pregnant, you will need to stop Study Drug immediately and permanently.

When you take the study drug home, keep it out of the reach of children or anyone else who may not be able to read or understand the label. Do not let anyone else take the study drug besides you.

It is possible that the researchers will learn something new during the study about the risks of being in it. If this happens, they will tell you about it. Then you can decide if you want to continue to be in this study or not. You may be asked to sign a new consent form that includes the new information if you decide to stay in the study.

Risks of the punch biopsy
Significant pain, bleeding, or infection are very rare with this type of punch biopsy. Although we may use a topical anesthetic on your skin so that you won't feel anything, you might feel some discomfort (like a scratch) when we give the medicine, and the next day. After your skin is healed, you might have a very small scar.

Will I benefit directly from the study?
This study is not designed to benefit you directly. Your CF may improve while you are in this study but it may not, and it may even get worse. This study is designed to learn more about whether cells from your skin can be used to predict which patients with CF might respond best to new drug therapies. The study results may be used to help others in the future.

Will I be compensated for my time and effort?

Travel, meals, accommodations, etc. for study visits will be paid for by the study and an incentive payment of $150 will be provided at Screening, Day 1, Day 14, Day 28 and Day 56. An incentive payment of $30 will be provided at the Day 7 telephone contact. For minors who participate in the study, travel expenses, etc. will also be provided for one parent and a single gift card for $150 per each visit will be provided to the parent (i.e. not the minor). For adult subjects who are accompanied by a caregiver, travel expenses, meals, and accommodations will also be provided for the caregiver. If an unscheduled visit is needed, an incentive payment of $150 will be provided. An unscheduled visit may occur if multiple procedures need to be repeated or if the punch biopsy needs to be performed on a day other than a scheduled visit. An incentive payment of $30 will be provided if the unscheduled visit is only for a repeat blood draw.

You will be asked to fill out a tax form, including your Social Security or Taxpayer Identification Number, in order to be reimbursed, depending on the amount and method of payment. Some payment methods involve mail coming to your house, which may be seen by others in your household. You can decline payment if you are concerned about confidentiality, or you can talk to the study team to see if there are other payment options.

What are my other options?

If you decide not to enter this study, there is care available to you outside of this research study. This includes all aspects of ongoing and standard care with your usual cystic fibrosis clinical team and care center. If you are taking other drugs such as Kalydeco™, Orkambi™ or Symdeko™ for CF, these may overlap and/or offer similar benefit to Trikafta™, and you will not be eligible for participation in the trial. You do not have to be in this study to be treated for cystic fibrosis.

Taking part in this study, may make you unable to participate in some other research studies, if they exclude people who have taken certain treatments. You should discuss this with the researchers if you have concerns. You may wish to research other study options at websites like clinicaltrials.gov, ResearchMatch.org and the CF Foundation website (cff.org).

How will you protect my private information that you collect in this study?

Whenever possible, a study number, rather than your name, will be used on study records. Your name and other identifying information will not appear when we present or publish the study results.

Certificate of Confidentiality

There is a Certificate of Confidentiality from the National Institutes of Health for this Study. The Certificate of Confidentiality helps us to keep others from learning that you participated in this study. Emory will rely on the Certificate of Confidentiality to refuse to give out study information that identifies you.

The Certificate of Confidentiality does not stop you or someone else, like a member of your family, from giving out information about your participation in this study. For example, if you let your insurance company know that you are in this study, and you agree to give the insurance company research information, then the investigator cannot use the Certificate to withhold this information. This means you and your family also need to protect your own privacy.

The Certificate does not stop Emory from making the following disclosures about you:

- Giving state public health officials information about certain infectious diseases,
- Giving law officials information about abuse of a child, elderly person or disabled person.
- Giving out information to prevent harm to you or others.
- Giving the study supporter or funders information about the study, including information for an audit or evaluation.

Storing and Sharing your Information

De-identified data from this study, including your de-identified genetic information, may be shared with the research community at large to advance science and health. Data from this study may be placed into public databases where, in addition to having no direct identifiers, researchers will need to sign data use agreements before accessing the data. We will remove or code any personal information that could identify you before your information is shared. This will ensure that, by current scientific standards and known methods, it is extremely unlikely that anyone would be able to identify you from the information we share. Despite these measures, we cannot guarantee anonymity of your personal data.

After you complete the follow-up visit, we will share your study data with your CF doctor who can help interpret the results.

Medical Record

If you have been an Emory patient before, then you already have a medical record with them. If you have never been an Emory patient, you do not have one. An Emory medical record will be made for you if an Emory provider or facility gives you any services or procedures for this study.

Copies of the consent form/HIPAA authorization that you sign will be put in any Emory medical record you have now or any time during the study.

Emory may create study information about you that can help with your care. For example, the results of study tests or procedures. These study results will be put in your Emory medical record. Anyone who has access to your medical records will be able to have access to all the study information placed there. The confidentiality of the study information in your medical record will be protected by laws like the HIPAA privacy rule. State and federal laws may not protect the research information from disclosure.

The results of some study tests and procedures will be used only for research purposes and will *not* be placed in your medical record. For this study, those items include: information resulting from your skin punch biopsy and/or blood sample used to test cell response to Trikafta™.

Tests and procedures done at non-Emory places may not become part of your Emory medical record. Also, if you decide to be in this study, it is up to you to let your other health providers know.

In Case of Injury

If you get ill or injured from being in the study, Emory will help you get medical treatment. Emory and the study supporter have not, however, set aside any money to pay you or to pay for this medical treatment. The only exception is if it is proven that your injury or illness is directly caused by the negligence of an Emory or study supporter employee. "Negligence" is the failure to follow a standard duty of care.

If you become ill or injured from being in this study, your insurer will be billed for your treatment costs. If you do not have insurance, or if your insurer does not pay, then you will have to pay these costs.

If you believe you have become ill or injured from this research, you should contact Dr. Sorscher at telephone number [redacted] or Dr. Linnemann at [redacted] You should also let any health care provider who treats you know that you are in a research study.

Costs

There will be no costs to you for participating in this study, other than basic expenses like transportation, which will be reimbursed after each visit. You will not be charged for any of the research activities. If the study procedures result in

any medical complications that would not fall under "injury" as discussed above, the cost of treatment for those complications may be charged to you or your insurance.

Withdrawal from the Study

You have the right to leave a study at any time without penalty. If you wish to leave the study, you should tell a member of the study staff by phone or in person and they will give you a form to complete (you should return the form to the study staff once it is complete) If you have to mail the form, please send it to:

Dr. Eric Sorscher
Department of Pediatrics
Emory University School of Medicine
[redacted]

If you choose to withdraw from the study, a study doctor will tell you how to stop taking the medications in a safe manner.

For your safety, however, you should consider the study Doctor's advice about how to go off the study treatment. If you leave the study before the final planned study visit, the researchers may ask you to have some of the final steps done.

The researchers also have the right to stop your participation in this study without your consent for any reason, especially if they believe it is in your best interest or if you were to object to any future changes that may be made in the study plan.

Authorization to Use and Disclose Protected Health Information

The privacy of your health information is important to us. We call your health information that identifies you, your "protected health information" or "PHI." To protect your PHI, we will follow federal and state privacy laws, including the Health Insurance Portability and Accountability Act and regulations (HIPAA). We refer to all of these laws as the "Privacy Rules." Here we let you know how we will use and disclose your PHI for the study.

PHI that Will be Used/Disclosed:

The PHI that we will use or share for the main research study includes:

- Medical information about you including your medical history and present/past medications
- Results of exams, procedures and tests you have before and during the study
- Laboratory test results

Purposes for Which Your PHI Will be Used/Disclosed:

We will use and share your PHI for the conduct and oversight of the research study. We will use and share your PHI to provide you with study related treatment and for payment for such treatment. We will also use and share your PHI to conduct normal business operations. We may share your PHI with other people and places that help us conduct or carry out the study, such as laboratories, data management centers, Institutional Review Boards (IRBs) and other study sites. If you leave the study, we may use your PHI to determine your health, vital status or contact information.

Use and Disclosure of Your Information That is Required by Law:

We will use and disclose your PHI when we are required to do so by law. This includes laws that require us to report child abuse or abuse of elderly or disabled adults.

Authorization to Use PHI is Required to Participate:
By signing this form, you give us permission to use and share your PHI as described in this document. You do not have to sign this form to authorize the use and disclosure of your PHI. If you do not sign this form, then you may not participate in the research study or receive research-related treatment. You may still receive non-research related treatment.

People Who will Use/Disclose Your PHI:
The following people and groups will use and disclose your PHI in connection with the research study:

- The Principal Investigator and the research staff will use and disclose your PHI to conduct the study and give you study related treatment.
- Emory may use and disclose your PHI to get payment for study related treatment and to run normal business operations.
- The Principal Investigator and research staff will share your PHI with other people and groups to help conduct the study or to provide oversight for the study.
- The National Institute of Health and The Cystic Fibrosis Foundation are the study supporters. The study supporters may use and disclose your PHI to make sure the research is done correctly and to collect and analyze the results of the research. The study supporters may disclose your PHI to other people and groups like study monitors to help conduct the study or to provide oversight for the study.
- The following people and groups will use your PHI to make sure the research is done correctly and safely:
    - Emory offices that are part of the Human Research Participant Protection Program and those that are involved in study administration and billing. These include the Emory IRB, the Emory Research and Healthcare Compliance Offices, and the Emory Office for Clinical Research
    - Government agencies that regulate the research including: Office for Human Research Protections; Food and Drug Administration
    - Public health agencies
    - Research monitors and reviewer
    - Accreditation agencies
    - The University of Alabama at Birmingham IRB, the oversite committee for the other enrolling site
- Sometimes a Principal Investigator or other researcher moves to a different institution. If this happens, your PHI may be shared with that new institution and their oversight offices. PHI will be shared securely and under a legal agreement to ensure it continues to be used under the terms of this consent and HIPAA authorization.

Expiration of Your Authorization
Your PHI will be used until this research study ends.

Revoking Your Authorization
If you sign this form, at any time later you may revoke (take back) your permission to use your information. If you want to do this, you must contact the study team at:

Dr. Eric Sorscher
Department of Pediatrics

Emory University School of Medicine
[redacted]

At that point, the researchers would not collect any more of your PHI. But they may use or disclose the information you already gave them so they can follow the law, protect your safety, or make sure that the study was done properly and the data is correct. If you revoke your authorization you will not be able to stay in the study.

Other Items You Should Know about Your Privacy

Not all people and entities are covered by the Privacy Rules. HIPAA only applies to health care providers, health care payers, and health care clearinghouses. If we disclose your information to people who are not covered by the Privacy Rules, including HIPAA, then your information won't be protected by the Privacy Rules. People who do not have to follow the Privacy rules can use or disclose your information with others without your permission if they are allowed to do so by the laws that cover them.

To maintain the integrity of this research study, you generally will not have access to your PHI related to this research until the study is complete. When the study ends, and at your request, you generally will have access to your PHI that we maintain in a designated record set. A designated record set is data that includes medical information or billing records that your health care providers use to make decisions about you. If it is necessary for your health care, your health information will be provided to your Doctor.

We may remove identifying information from your PHI. Once we do this, the remaining information will not be subject to the Privacy Rules. Information without identifiers may be used or disclosed with other people or organizations for purposes besides this study.

Contact Information

Contact Eric Sorscher at [redacted] or Rachel Linnemann at [redacted]:

- if you have any questions about this study or your part in it,
- if you feel you have had a research-related injury or a bad reaction to the study drug, or
- if you have questions, concerns or complaints about the research

Contact the Emory Institutional Review Board at 404-712-0720 or 877-503-9797 or irb@emory.edu:

- if you have questions about your rights as a research participant.
- if you have questions, concerns or complaints about the research.
- You may also let the IRB know about your experience as a research participant through our Research Participant Survey at http://www.surveymonkey.com/s/6ZDMW75.

Optional Procedure for persons with N1303K:

Would you like to provide a skin biopsy? You may still participate in the study without providing a skin biopsy. Please write your initials next to your choice below:

_____ YES, I would like to provide a skin biopsy

_____ NO, I would not like to provide a skin biopsy

Consent and Authorization

TO BE FILLED OUT BY SUBJECT ONLY

Please **print** your name, **sign**, and **date** below if you agree to be in the main study. By signing this consent and authorization form, you will not give up any of your legal rights. We will give you a copy of the signed form to keep.

_____

Name of Subject

_____ _____

Signature of Subject (18 or older and able to consent) **Date** **Time**

_____ _____

Signature of Legally Authorized Representative with authority for research decisions **Date** **Time**

_____

Authority of Legally Authorized Representative or Relationship to Subject

TO BE FILLED OUT BY STUDY TEAM ONLY

_____

Name of Person Conducting Informed Consent Discussion

_____ _____

Signature of Person Conducting Informed Consent Discussion **Date** **Time**