Informed Consent Cover Page for FDAAA consent posting:

Official Title:	A Phase I/II, Intra-Patient Dose-Escalation Study of the Selective GlyT1 Inhibitor, Bitopertin for Steroid-Refractory Diamond-Blackfan Anemia.
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PRINCIPAL INVESTIGATOR: David J. Young, M.D., Ph.D.

STUDY TITLE: A Phase I/II, Intra-Patient Dose-Escalation Study of the Selective Glyt1 Inhibitor, Bitopertin for Steroid-Refractory Diamond-Blackfan Anemia.

STUDY SITE: NIH Clinical Center (NIH CC)

Cohort: Standard Consent

Consent Version: March 4, 2024

WHO DO YOU CONTACT ABOUT THIS STUDY?

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RESEARCH NURSE: Bretagne Cowling, R.N.**;** bretagne.cowling@nih.gov, 301-827-3479

KEY INFORMATION ABOUT THIS RESEARCH

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). This section provides the information we believe is most helpful and important to you in making your decision about participating in this study. Additional information that may help you decide can be found in other sections of the document. Taking part in research at the NIH is your choice.

You have Diamond-Blackfan Anemia (DBA), and your doctors have determined that the standard treatment has not been effective for you, or the side effects are making it dangerous to continue treatment.

The purpose of this study is to see if a new drug called bitopertin can increase your blood counts and/or reduce the number of transfusions you need. Bitopertin is considered an investigational drug, which means that it is a drug that is not Food and Drug Administration (FDA)-approved for use outside of research studies.

On your first visit we will ask you to come to the NIH and run some tests to make sure you are eligible for the study. This is called the screening visit. If we find that you are eligible, then main study consists of visits every 4 weeks that can take place either at NIH or remotely via telehealth. If done remotely, we will ask you to have labs done at your local laboratory provider and to forward a copy of the results to the NIH. We may also ask you to ship a blood sample to the NIH for specialized testing. We will provide you with instructions on how to do that. At each visit we will assess your body's response to bitopertin and monitor for side effects.

The main part of the study consists of two phases. In the first phase (Weeks 0 to 20), we will ask you to start 5 mg/day bitopertin pill once a day for four weeks. Based on your response and tolerance level over this period, we will continue to increase the dose every 4 weeks up to 60

mg/day (5 mg, 10 mg, 20 mg, 40 mg, and 60 mg) until the effective and/or tolerable dose for you is identified.

In the second phase, we will ask you to continue taking the recommended dose until the Month 8 visit (32 weeks). We may adjust that dose if you experience side effects or if your blood counts change.

We will also ask you to have additional research procedures (see the events & procedure tables). If you agree, you will be invited for an optional visit at Month 14.

While taking bitopertin, if you experience any new and concerning symptoms, you should immediately notify your doctor and us.

Even if you have telehealth visits for certain visits, you will need to return to the NIH Clinical Center at the end of approximately eight months (32 weeks) for blood tests and bone marrow biopsy to see how your bone marrow is responding to bitopertin.

The study staff will provide specific information such as dates and times for your study visits and tests.

If you show response and tolerate bitopertin at the 8-month visit, you will be invited to continue on an extended phase of the bitopertin study, lasting for up to an additional three (3) years, unless you decide that you no longer want to be in this study, or the study is stopped for safety or other issues. Participating in this "extended phase" is optional, and we will discuss the risks and benefits for that at a later visit. If you choose not to continue on the extended phase of the study, we may still ask you to come back 6 months after your month 8 visit. Whether you return for this visit or not, we will reach out to you about one month after your 8-month visit to follow-up on how you have been doing off of the drug. For patients on higher doses, this extended phase will be the first experience with humans taking up to 60 mg once daily for longer than 4 months.

Potential known risks related to this research study include:

From the study procedures: pain, discomfort, and bruising are the most frequent complaints at the site of the bone marrow biopsy or blood draw, with some patients experiencing bleeding or rare infections.

From bitopertin: side effects may include headache, dizziness, blurred vision, dry skin, nausea, and fatigue.

You might not benefit from this study. DBA patients may respond to bitopertin leading to an improvement in blood cell numbers and reduced number of transfusions.

Instead of being in this study, you may choose to continue your current treatment by your home health care provider.

There may be other alternative experimental therapies available at other research centers or that may become available at a later date. You cannot participate in this study at the same time you are on other experimental treatments for Diamond-Blackfan Anemia.

The remaining document will now describe the research study in more detail. This information should be considered before you make your choice. Members of the study team will talk with you

about the information in this document. Some people have personal, religious, or ethical beliefs that may limit the kinds of medical or research interventions in which they would want to participate. Take the time you need to ask any questions and discuss this study with NIH staff, and with your family, friends, and personal health care providers.

If the individual being asked to participate in this research study is not able to give consent for themselves, you, as the Legally Authorized Representative, will be their decision-maker and you are being asked to give permission for this person to be in this study. For the remainder of this document, the term "you" refers to you as the decision-maker and/or the individual being asked to participate in this research.

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 purpose of this research study is to test if a new drug called bitopertin can increase blood counts and/or reduce the number of transfusions for Diamond-Blackfan Anemia patients who have not responded or tolerated previous therapies such as steroids.

We are asking you to join this research study because you have been diagnosed with Diamond-Blackfan Anemia, and steroid therapy is no longer considered the best therapy for you.

Bitopertin is considered investigational, which means that it has not been approved by the U.S. Food and Drug Administration (FDA) to treat Diamond-Blackfan Anemia. We are testing it in this research study to see if bitopertin will help treat patients with Diamond-Blackfan Anemia by increasing their blood counts.

WHAT WILL HAPPEN DURING THE STUDY?

If you decide to take part in this study, you will be asked to take bitopertin pill once a day for eight months (32 weeks).

If you are of childbearing age, you must agree to use a highly effective method of contraception for the duration of the study and for 30 days after the last dose of the study drug. The study team will discuss with you the acceptable methods of contraception.

We will first ask you to come to the NIH to confirm that you are eligible for the study. This is called the screening visit. We will run some lab tests, check your medical history, do a physical exam.

As part of this study, we will test you for infection with the human immunodeficiency virus (HIV), the virus that causes AIDS. If you are infected with HIV you will still be able to participate in this study. We will tell you what the results mean, how to find care, how to avoid infecting others,

how we report HIV infection, and the importance of informing your partners at possible risk because of your HIV infection.

If we find that you are eligible for the study, we will ask you to come back to start the study.

To find the dose that works best for your disease, first we will start at low dose of the drug and increase it every 4 weeks for up to 20 weeks, and then at a steady dose for 12 weeks. We will monitor you every month for side effects and for the presence of immature red blood cells called reticulocytes which may offer early clues as to how your bone marrow is responding to the drug. If you either experience a side effect or your reticulocyte count is high, we will stop increasing the amount of drug we give you and you will remain on the dose that did not give you bad side effects until the end of the 32 weeks.

The following tables list the events and procedures that you will have at each visit.

Visit/Time Point	Consent visit (Screening*)	Day 0*	Week (± 10 days) – Telehealth Visits							Month 8	Month 14 Off-study (optional)
			4	8	12	16	20	24	28		
Clinical Assessments											
Medical history	X	X								X	X
Physical examination	X									X	X
PHQ-8 and C-SSRS questionnaires	X									X	
Concurrent medication review	X	X	X	X	X	X	X	X	X	X	X
Transfusion records review	X	X	X	X	X	X	X	X	X	X	X
Blood Draw	X		X	X	X	X	X	X	X	X	X
Electrocardiogram (ECG)	X	X									
Pregnancy testing	X	X								X	
Bone marrow aspiration and core biopsy	X									X	X
Genetic sequencing	X										
Health-Related Quality of Life (HRQL) questionnaire		X								X	X
Pill count										X	

*Some tests and procedures do not need to be repeated at the discretion of the study team if recent results are available
All participants will be contacted 30 days after stopping drug to review any events that may have occurred in the intervening time.

Telehealth visits: If a visit is done via telehealth, we will ask you to have labs done at your local laboratory provider and to forward a copy of the results to the NIH. We may also ask you to ship a blood sample (3 mL or about half teaspoon) to the NIH for specialized testing. We will provide you with instructions on how to do that.

Procedure Name	Procedure Description
 Medical History, Transfusion records and Concurrent Medication Review	As part of this study, we will collect some information from your medical record. This will include information such as your age, sex, race, height and weight, pain level, medications you are taking and the results of any laboratory or diagnostic tests.
 Physical Examination	You will have a physical exam that includes, vital signs, height, weight, and BMI.
 Blood Draw	You will have blood drawn from a vein. This may require a needle stick in your arm. The amount of blood we will take is about 4-5 tablespoons during your NIH visits, and 1-2 tablespoons a month at home.
 Electrocardiogram (ECG)	An electrocardiogram (ECG) is a test that is performed while you lie still for about 5 minutes. It involves placing electrodes (small stickers that are attached to wires that go to the machine) on the chest and arms/legs and recording the electrical activity of your heart. If you have a lot of hair on your chest, it may hurt a little bit when they remove these stickers.
 Pregnancy Testing	If you are female and can get pregnant, you will have a blood or urine pregnancy test at each NIH study visit except the last one.
 Bone Marrow aspiration & Core biopsy	Because Diamond-Blackfan Anemia affects your blood, we will need to take samples of your bone marrow (where blood is produced). We will numb the area above your hip bone with a local anesthetic. The anesthetic can cause some temporary stinging and burning. When the area is numb, we will remove a sample of the bone and marrow with a thin needle. You may feel a pulling sensation and discomfort as the marrow is withdrawn. The amount of marrow taken is very small and will not further compromise your body's ability to form blood.

Procedure Name	Procedure Description
	A pathologist will read the sample to confirm your diagnosis.
 Questionnaires	We will ask you to complete a series of questionnaires that will take up to 60 minutes to complete. We will ask you to complete a Health-Related Quality of Life (HRQL) questionnaire at each NIH visit which will ask you about the symptoms caused by DBA and how it affects your daily life and activities. We will also ask you to complete the PHQ-8 and C-SSRS Questionnaires at Screening Visit and Month 8 visit to check for any concerns that could limit your participation in this study.
 Pill Count	At interval in-person evaluations at the NIH, you will be asked to return unopened containers and unused tablets for drug accounting and, if needed (end-of-study), disposal.
 Drug Diary	You will be provided drug diary to record dose and administration during the first 8 months (32 weeks) of study participation. This diary will be reviewed at telehealth and in-person NIH visits.
 Genetic Sequencing	Your blood and tissue samples contain genes, which are made up of DNA (deoxyribonucleic acid) which serves as the "instruction book" for the cells that make up our bodies. Sequencing of your genes will determine the exact order of the base pairs (chemical letters) in the blood and tissue samples. We will only ask for this test if genetic testing results from a previous assessment at the NIH or outside are **not** available. This test will assess changes to genes specific to DBA and will allow us to confirm the diagnosis of DBA (occasionally some diseases can strongly mimic DBA) and possibly identify the changes that are causing your disease.

HOW LONG WILL THE STUDY TAKE?

If you agree to take part in this study, your involvement is expected to last for up to three (3) years and eight (8) months. This consent is for the first 8 months (32 weeks) of your involvement, and the optional return visit at 14 months. If, after the 8-month visit, you are eligible and choose to enroll on the additional 3-year "extended phase", we will discuss the risks and benefits of doing so at that time, and you will sign a separate consent.

HOW MANY PEOPLE WILL PARTICIPATE IN THIS STUDY?

We plan to have approximately 25 eligible people participate in this study at the NIH.

WHAT ARE THE RISKS AND DISCOMFORTS OF BEING IN THE STUDY?

Risks related to bitopertin

Likely side effects:

No side effects have been reported as occurring in more than 10% of patients.

Less likely (occurring in less than or equal to 10% of patients):

Dizziness, headache, blurred vision, dry skin, nausea, fatigue.

Also reported in some patients:

Nasopharyngitis (nose and throat irritation), insomnia (sleep loss), anxiety, depression

Extended-phase dosing:

Patients who respond at the highest dose (60 mg per day) will have already demonstrated tolerance of this bitopertin dose for four (4) months. However, there is no previous human experience for 60 mg once daily dosing longer than four (4) months. There may be unknown side effects or less frequent side effects may occur more frequently during prolonged treatment at this dose that were not seen at lower doses.

Risks related to blood draw

Blood draws may cause pain, redness, bruising or infection at the site of the needle stick. Rarely some people faint.

Risks related to bone marrow biopsy

The bone marrow aspiration and biopsy may cause pain, bruising, bleeding and infection. Soreness near the site may last for a couple of days after the procedure. You may have more pain, risk of bleeding and bruising if you complete both aspiration and biopsy rather than just the aspiration. If your pain is severe or you develop a fever, please contact the study team immediately.

Risks related to electrocardiogram

Other than possibly experiencing some minor skin irritation from the electrodes, there are no anticipated risks related to complete the electrocardiogram and/or the echocardiogram.

Risks related to questionnaire/survey

Some of the questions in the questionnaire may be upsetting or make you feel uncomfortable. You can skip any of the questions you do not want to answer, and you can stop at any time.

Risks related to genetic testing

Psychological or social risks associated with return of secondary findings

As part of the research study, it is possible that you could learn that you have genetic risks for another disease or disability. This may be upsetting and, depending on what you learn, might create a need to make challenging decisions about how to respond.

Although your genomic information is unique to you, you share some genomic similarities with your children, parents, brothers, sisters, and other blood relatives. Therefore, learning your research results could mean something about your family members and might cause you or your family distress. Before joining the study, it may be beneficial to talk with your family members about whether and how they want you to share your results with them.

Protections against misuse of genetic information

This study involves genetic testing on samples. Some genetic information can help predict future health problems of you and your family and this information might be of interest to your employers or insurers. The Genetic Information Nondiscrimination Act (GINA) is a federal law that prohibits plans and health insurers from requesting genetic information or using genetic information. It also prohibits employment discrimination based on your health information. However, GINA does not address discrimination by companies that sell life insurance, disability insurance, or long-term care insurance. GINA also does not protect you against discrimination based on an already-diagnosed condition or disease that has a genetic component.

What are the risks related to pregnancy?

If you are able to become pregnant, we will ask you to have a pregnancy test before starting this study. You must use effective birth control methods and try not to become pregnant while participating in this study and for 30 days after the last dose of bitopertin. If you become pregnant, there may be unknown risks to the fetus or unborn child, or risks that we did not anticipate. There may be long-term effects of the treatment being studied that could increase the risk of harm to a fetus. You must tell the study doctor if your birth control method fails while you are in the study. If you think or know you have become pregnant while participating in this research study, please contact the study team as soon as possible. If you plan to become pregnant in the future, please discuss with the study team how long you need to wait before becoming pregnant after completing the study.

If you are a sexually active person with a partner able to become pregnant, it is important that your partner not become pregnant during your participation in this study. There may be unknown risks to a fetus or risks we did not anticipate. You and your partner must agree to use effective birth control methods for the duration of the study and for 30 days after the last dose of bitopertin if you want to take part in this study. If you think your partner has become pregnant during your participation in this study, please contact the study team as soon as possible. If you and your partner plan for your partner to become pregnant after your participation in this study, please discuss this with the study team.

WHAT ARE THE BENEFITS OF BEING IN THE STUDY?

You might not benefit from being in this study. However, the potential benefit to you might be that you may respond to bitopertin leading to an improvement in blood cell numbers and reduced number of transfusions.

Are there any potential benefits to others that might result from the study?

In the future, other people might benefit from this study because it will help doctors understand how bitopertin affects DBA. It will also give doctors new and better information into how the

changes seen in DBA cause the anemia and how it responds to drugs such as bitopertin. Even if this trial is unsuccessful, the information we gain may help researchers design future drugs and trials to treat DBA.

WHAT OTHER OPTIONS ARE THERE FOR YOU?

Before you decide whether or not to be in this study, we will discuss other options that are available to you. Instead of being in this study, you could choose to be treated by your home health care provider or participate in other research studies.

DISCUSSION OF FINDINGS

New information about the study

If we find out any new information that may affect your choice to participate in this study, we will get in touch with you to explain what we have learned. This may be information we have learned while doing this study here at the NIH or information we have learned from other scientists doing similar research in other places.

Return of research results

You will be provided with the results of the standard clinical labs, but the results of our research studies will not be provided to you or your referring doctor.

If we find a genetic change that may be of clinical significance from the bone marrow disease genes we are interested in, we will discuss what it means to have this change and refer you to a genetic counselor if needed.

EARLY WITHDRAWAL FROM THE STUDY

You may withdraw from study at your request. The risks of withdrawing will be discussed, as will alternative treatment options. If you choose to withdraw while taking bitopertin, we will strongly encourage you to continue to have your labs monitored until you initiate an alternative therapy.

You may also be taken off study prior to the completion of the study for the following reasons:

- Your study doctor decides that continuing in this study would be harmful to you
- You are diagnosed with other condition other than DBA
- You start another treatment other than the study drug (bitopertin)
- You do not keep appointments or do not wish to take the study drug as instructed
- We are unable to reach you

If you become pregnant while taking the study drug, we will ask you to stop taking the study drug immediately. If you agree, you will be monitored until the completion of the pregnancy and taken off of the study then.

STORAGE, SHARING AND FUTURE RESEARCH USING YOUR SPECIMENS AND DATA

Will your specimens or data be saved by the study team for use in other studies?

As part of this study, we are obtaining specimens and data from you. We plan to store and use these specimens and data for studies other than the one described in this consent form that are going on right now, as well as studies that may be conducted in the future. The specimens and data will be kept in a way that we will still know that they came from you (i.e., they will be identifiable to us). If we use your identifiable specimens or data for future research, our study will be reviewed and approved by an Institutional Review Board who will make sure that we are protecting your confidentiality. These future studies might help us better understand Diamond-Blackfan Anemia or other diseases or conditions. This could include studies to develop other research tests, treatments, drugs, or devices, that may lead to the development of a commercial product by the NIH and/or its research or commercial partners. There are no plans to provide financial compensation to you if this happens. Also, it is unlikely that we will learn anything from these studies that may directly benefit you.

I give permission for my identifiable specimens and data to be stored and used by the study team for future studies as described above.

_____ Yes _____ No

Initial Initial

Will your specimens or data be shared with other researchers for use in other studies?

We may share your specimens and data with other researchers. The other researchers may be doing studies in similar areas to this study or in other unrelated areas. These researchers may be at NIH, other research centers and institutions, or at commercial entities.

One way that we may share your data is by putting it into a large database called a repository, which is a way to make it widely available to the research community. If we do place your data in a repository, it will be labeled with a code, (not with your name or other information that could be used to easily identify you). Even though it will only be labeled with a code, some types of data, in particular data about your genes (called genetic or genomic data), can be used to figure out who you are, although this is difficult to do, and we think it is unlikely to happen.

The data in the repository will only be available to qualified researchers. These researchers must receive permission before they are allowed to access the data. Before receiving the data, the researchers must promise that they will not try to figure out the identity of the research participants.

If we do share your specimens or data, we will know that the specimens and data came from you. However, the other researchers will not know that they came from you (i.e., they will be de-identified).

I give permission for my **de-identified** specimens and data to be shared with and used by other researchers for future studies.

_____ Yes _____ No

Initial Initial

Information about all the people (including you) in this study may be combined to create what is called summary information. The summary information may be placed in a database and will be made available to researchers only if they are granted permission. However, the summary information may still be shared in scientific publications without permissions. This information will help the researchers understand if some patterns are more common than others among everyone who was a part of this study. The risk of anyone identifying you based on this information is very low.

In addition to the planned use and sharing described above, we might remove any labels from your specimens and data that might identify you (i.e., anonymize them), and use them or share them with other researchers for future studies at the NIH or other places. When we or the other researchers use your anonymized specimens and data for these projects, there will be no way to know that they came from you. We want to make sure that you understand that this is a possibility if you participate in this study. Once we do this, we would not be able to remove your specimens or data from these studies or prevent their use in future studies because we would not be able to tell which specimens or data belong to you.

Risks of storage and sharing of specimens and data

When we store your specimens and data, we take precautions to protect your information from others who should not have access to it. When we share your specimens and data, we will do everything we can to protect your identity, for example, when appropriate, we remove information that can identify you. Even with the safeguards we put in place, we cannot guarantee that your identity will never become known, or that no one will gain unauthorized access to your information. New methods may be created in the future that could make it possible to re-identify your specimens and data.

Can you change your mind about use and sharing for future research?

If you change your mind and do not want us to store and use your specimens and data for future studies, you should contact the study team. We will do our best to comply with your request but cannot guarantee that we will always be able to destroy your specimens and data. For example, if some research with your specimens and data is already complete, the information from that research may still be used. Also, if the specimens and data have been shared already, it might not be possible to withdraw them.

How long will your specimens and data be stored by the NIH?

Your specimens and data may be stored by the NIH until they are no longer of scientific value.

PAYMENT

Will you receive any type of payment for taking part in this study?

You will not receive any payment for taking part in this study.

REIMBURSEMENT

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

This study offers reimbursement or payment for travel and lodging while participating in the research. The amount, if any, is guided by NIH policies and guidelines.

Participants will be reimbursed a total of $80 per day for hotel. Long-distance travel will be reimbursed at the government airfare cost, or for a train or bus of the same or lesser value. Receipts must be provided for all expenses.

If your travel to the NIH Clinical Center (e.g., flight, hotel) is arranged and paid for by the NIH, the agency making the reservations and their representatives will have access to your identifiable information.

COSTS

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. Study drug will be provided to you at no cost. All study required procedures performed at the National Institutes of Health Clinical Center will be at no cost to you. If testing beyond what is routinely required for clinical monitoring needs to be done outside of NIH for the study, then NIH may arrange to have this done free of charge. The NIH will arrange for the shipment of any samples at no cost to you.

CONFLICT OF INTEREST (COI)

The NIH reviews NIH staff researchers at least yearly for conflicts of interest. This process is detailed in a COI Guide. You may ask your research team for a copy of the COI Guide or for more information. Members of the research team who do not work for NIH are expected to follow these guidelines or the guidelines of their home institution, but they do not need to report their personal finances to the NIH.

The NIH and the research team for this study are using Bitopertin developed by ThermoFisher (on behalf of Disc Medicine) through a collaboration between the study team and the company. The company also provides financial support for this study.

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 study Sponsor the National Heart, Lung, and Blood Institute (NHLBI) Office of Clinical Director (OCD) at the NIH or their agent(s)
- Qualified representatives from Disc Medicine, the pharmaceutical company who distributes Bitopertin.

The researchers conducting this study and the NIH follow applicable laws and policies to keep your identifying information private to the extent possible. However, there is always a chance that, despite our best efforts, your identity and/or information about your participation in this research may be inadvertently released or improperly accessed by unauthorized persons.

In most cases, the NIH will not release any identifiable information collected about you without your written permission. However, your information may be shared as described in the section of this document on sharing of specimens and data, and as further outlined in the following sections.

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 information that 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 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.

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, about your rights as a research participant, or about any research-related injury, contact the Principal Investigator, David J. Young, M.D., Ph.D., david.young2@nih.gov, Phone: 301-827-7823. Other researchers you may call are: Bretagne Cowling, R.N., at 301-827-3479. 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

Legally Authorized Representative (LAR) for an Adult Unable to Consent: I have read the explanation about this study and have been given the opportunity to discuss it and to ask questions. I am legally authorized to make research decisions on behalf of the adult participant unable to consent and have the authority to provide consent to this study. As applicable, the information in the above consent was described to the adult participant unable to consent who agrees to participate in the study.

Signature of LAR	Print Name of LAR	Date

Investigator:

Signature of Investigator	Print Name of Investigator	Date

Witness should sign below if either:

1. **A short form consent process has been used to enroll a non-English speaking subject or**
2. **An oral presentation of the full consent has been used to enroll a blind or illiterate subject**

Signature of Witness	Print Name of Witness	Date

NIH ADMINISTRATIVE SECTION TO BE COMPLETED REGARDING THE USE OF AN INTERPRETER:

____ An interpreter, or other individual, who speaks English and the participant's preferred language facilitated the administration of informed consent and served as a witness. The investigator obtaining consent may not also serve as the witness.

____ An interpreter, or other individual, who speaks English and the participant's preferred language facilitated the administration of informed consent but did not serve as a witness. The name or ID code of the person providing interpretive support is: _____.