PRINCIPAL INVESTIGATOR: Danielle E. Pregent-Arnold, MD

STUDY TITLE: A Phase II Study of Allogeneic Hematopoietic Stem Cell Transplantation with Briquilimab-Based Conditioning in Participants with GATA2 Deficiency

STUDY SITE: NIH Clinical Center

Cohort: Affected Patient

Consent Version: 07/29/2025

WHO DO YOU CONTACT ABOUT THIS STUDY?

Danielle E. Pregent-Arnold, M.D. by phone at 240-281-3922 or email: danielle.arnold@nih.gov

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.

We are asking you to take part in this study because you have a disorder of the immune system called GATA2 deficiency that has caused significant health problems.

When hematopoietic stem cells (HSCs) are taken from one person (called the "donor") and given to another person (called the "recipient"), it is known as a transplant.

The purpose of this study is to see if a HSC transplant from another person in combination with a new drug Briquilimab can be successfully done in people with GATA2 deficiency.

Stem cells are immature cells like seeds that grow in the bone marrow and make all of the cells needed for normal blood (red blood cells, white blood cells, and platelets) and immunity, the body's defense against infections, cancers, and other insults. Some of these immune cells hopefully also will attack the recipient's immune cells that don't work and try to get rid of them.

Transplanting HSCs from someone else is a commonly used treatment for other blood diseases and involves the transfer of HSCs from a person with a healthy immune system to a person with a disease or cancer. Stem cells are collected for transplantation by taking samples of blood from the donor and given to you through a plastic tube in the veins.

The transplant involves "conditioning" necessary to decrease the number of your own stem cells and prepare your body to accept HSCs from another person.

To prepare your immune system to accept transplant, we will use a new drug Briquilimab in combination with fludarabine and total body irradiation (TBI). Depending on the donor, we may use an additional drug, cyclophosphamide. This is called "conditioning".

Briquilimab is an antibody designed to make a space in your bone marrow for new stem cells.

To prevent graft-versus-host disease (GVHD), a condition in which the donor cells attack your own normal cells, we will give you cyclophosphamide, tacrolimus, and mycophenolate mofetil after transplant.

Fludarabine, cyclophosphamide, mycophenolate mofetil, tacrolimus, and TBI are commonly used in other treatments for transplant as a standard of care.

Briquilimab is being used in other clinical trials but has not been approved by the U.S. Food and Drug Administration (FDA). Therefore, the use of Briquilimab, fludarabine, cyclophosphamide, mycophenolate mofetil, tacrolimus, and TBI in this study is considered investigational. However, the FDA has given us permission to use Briquilimab in this study.

While transplants are commonly used to treat immune diseases, cancers, and diseases of the blood, transplant is a treatment with a lot of risk and complications, including complications that can lead to death. We hope that a transplant will improve your symptoms. Transplant does have more severe side effects than if you were to receive standard care. In the first 2-4 weeks after transplant, nearly all participants will have very low blood counts. Engraftment – when the donor's stem cells start making blood cells for you – usually occurs between days +12 and +28 after transplant. There is a small chance (5 to 10 people out of 100 may have this) that you may reject your donor's stem cells. If that were to happen, your own blood cells would likely recover, and you would then decide whether to receive standard therapy or proceed with another transplant. **However, there is a possibility your own blood cells may not recover, and you would need another transplant either from the same or a different donor in an urgent fashion to decrease your risk of infection, bleeding, and death**. During the time before the second transplant, if needed, we would attempt to support you with transfusions, growth factors, and antibiotics.

If you prefer, you can continue your current treatment and this can be prescribed/given by your regular doctor, instead of taking part in this study.

If you decide to join this study, here are some of the most important things that will happen:

- First, we will find out if you can take part in this study. This will involve evaluation of your health history, physical exam, having standard blood tests and tests to check your heart and lung function, and a bone marrow biopsy. We also need to confirm that you have mutations in the GATA2 gene, so we will collect an additional blood sample to do the test. To look at your GATA 2 gene, we will use test called "Sanger sequencing." This where we will do special test in the lab to look at the entire sequence, or order, of GATA2 gene and see if there is a mutation that causes your disease.
- Before the transplant, we will perform standard tests to find out if you can continue in the study (if some of these were done during screening, they may not need to be repeated). To undergo the transplant, it is best if your inflammation and any infections are well controlled and your lungs, liver, kidneys, and heart function as well as possible

since transplant is difficult on your body. Members of the study team will work with you and with your other doctors to see if and when is the best time for you to receive the transplant on this study.

- You will be admitted to the NIH Clinical Center at the beginning of conditioning and continue to be hospitalized until you no longer require intravenous fluids or medications and are strong enough to carry out your daily activities. This usually takes about 4 weeks from the time of transplant but may be longer.
- Depending on the available donor, you will be assigned to Group 1 if your donor is fully matched (conditioning with Briquilimab, fludarabine, and TBI) or Group 2 if your donor is partially matched (conditioning with Briquilimab, fludarabine, cyclophosphamide, and TBI). After conditioning, which takes about 2 weeks, you will receive the transplant. All drugs and cells will be given to you by intravenous (IV) infusion.
- After you are released from the Clinical Center, you will need to stay within a 1-hour drive from the NIH for a minimum of 100 days after transplant and have an adult caregiver with you during this time. This stay may be longer if there are complications. During this time, we need to see you at the NIH at least once per week.
- To assess your health and to determine what impact, if any, the transplant had on your disease, we will need to continue to see you at the NIH Clinical Center approximately 180 days after the transplant and yearly for three years.
- For a few months after your transplant, your immune system will be weak, and you will have to avoid or modify certain activities (including school and work, public gyms, encounters with any large group of people, interactions with children), and take extra precautions to avoid infections. You will need frequent blood tests to follow your organ function, blood counts, and drug levels, as well as check for infection. We will also collect your blood for research purposes when you come for your main timepoint follow-up visits.
- After the transplant, you are at risk of complications such as infections, graft-versus-host disease, bleeding, and organ damage. It is very important to take medications as instructed by the study doctors to help prevent these complications that could be dangerous and lead to severe health problems. There may be additional tests, procedures, or therapies that are not outlined in the consent that the study team may recommend if a complication of transplant occurs. However, some of these complications may not be treatable or may cause death. Moreover, your disease could come back or get worse. Additionally, this treatment may result in the inability to have children in the future unless you store sperm or eggs prior to transplant.
- You must use effective birth control methods and try not to become pregnant or father a child before study treatment and for 1 year after transplant. If the transplant is not done, you must avoid making someone pregnant for 4 months after you finish chemotherapy if you are male. If you are female and the transplant is not done, you should avoid becoming pregnant for 12 months after you finish chemotherapy.

- By taking part in this study, you may benefit from the new immune system created from the transplant. If your immune system functions normally, it is likely that you would have improved peripheral blood cell counts, fewer infections and decreased risk of myelodysplastic syndrome and leukemia. We cannot predict whether you will have this benefit or not.

You are free to stop participating in the trial at any time. However, because you need to be followed closely for several months to years after transplant for safety, we advise you to fully understand the requirements to undergo a transplant and the extent and length of commitment before deciding to take part. If you receive conditioning, you should not withdraw from the study without receiving donor cells; if you receive the donor cells, you should try not to withdraw without receiving the medications to prevent graft-versus-host disease and infection. If you decide to stop, the study doctor may ask you to agree to certain tests to make sure it is safe for you to stop.

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 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?

This is a research study. The purpose of this study is to see if a blood transplant from another person in combination with a new drug Briquilimab can be successfully done in people with GATA2 deficiency.

There are other ways to do a transplant for patients with GATA2 but we believe that using Briquilimab will have less side effects than using standard conditioning agents.

The reason for the transplant in this study is to provide a new, healthy immune system to try to improve your disease.

The role of a healthy immune system is to attack foreign invaders like bacteria or other infections or to recognize and kill cells that have "gone bad," like cancer cells. Often after a transplant, one type of the donor's cells, T cells, (from the new immune system) can see your (the recipient's) body as "foreign", and this can lead to graft-versus-host disease (GVHD). GVHD is when the donor's immune system attacks the recipient's body (usually the skin, liver, and/or gut) as if it is a

foreign invader or bad cell. This complication can make the transplant unsuccessful. We will give you drugs after the transplant to prevent GVHD and will monitor you for symptoms.

WHAT WILL HAPPEN DURING THE STUDY?

Before you begin the study

Before beginning the study, you will need to undergo tests and/or procedures to help your doctor verify whether you can participate. This is called screening. Most of the exams, tests, and procedures you will have are part of the usual approach for your disease. However, there are some extra procedures that you will need to have if you take part in this study. If you have already undergone some of these examinations very recently, your doctor may decide not to repeat them. Briefly, these tests include:

- A review of any past or current medical conditions and medications you are taking
- Physical examination, including weight, height, and vital signs
- Review of your symptoms, and your ability to perform your normal activities
- Routine blood tests to find out if you are anemic, have low blood counts, and if your liver and kidneys, are working well (~1 tablespoon of blood)
- If you are an adult, your urine will be collected for 24 hours
- Blood or urine test if you are a woman or a girl who can have children
- Hepatitis B, Hepatitis C, and HIV testing (~2 tablespoons). As part of this study, we will test you for infection with Hepatitis B, C, and HIV, the virus that causes AIDS. If you are infected with HIV, you will not be able to take part in this study. We will tell you what the results mean, how to find care, how to avoid infecting others, how we report HIV infections, and the importance of informing your partners at possible risk because of your HIV infection
- Echocardiogram – ultrasound of your heart to evaluate your heart
- Electrocardiogram (EKG) - a test of the electrical activity of your heart
- Pulmonary function tests (PFTs) – you will need to breathe in and out of a machine, which measures how well your lungs are working
- Bone Marrow Aspiration / Biopsy: You will be asked to have a bone marrow biopsy and aspiration to collect bone marrow tissue and cells from your hip. Bone marrow is the soft material in the center of bones that produces new blood cells. The area will be numbed with lidocaine and, once numb, a large needle will be inserted through a small cut to draw about 4 tablespoons of marrow out of the bone and to possibly remove a small piece of bone. Your level of pain will be monitored throughout the procedure, and you'll be encouraged to voice any concerns. Additional numbing medicine or sedation may be utilized if necessary. The entire procedure will take about 1 hour to complete
- Blood typing for donor selection/matching (~2 tablespoons of blood)
- Meeting with a liver specialist (only if you were infected Hepatitis B or Hepatitis C)

- Evaluation of GATA2 mutation status. This is not a standard clinical care test, this is a research test needed for this treatment (~1 tablespoon of blood).

You will be removed from the study if the tests show that you are not able to participate, or we cannot find a suitable donor for you.

During the study

Before your transplant, you will have a central venous catheter put into a vein in your chest or neck. Medications and transfusions can be given through the catheter and blood can be drawn from the catheter during your transplant. This should make blood draws easier and less painful. There is a chance that the catheter can become infected, clogged, or moved out of the correct position. Sometimes, in these cases, the catheter can be fixed or treated; other times, it may need to be replaced. You or your caregiver will be responsible for caring for the catheter if you continue to need it once discharged from the hospital. The nursing staff will teach you how to care for the catheter.

Tests before conditioning and cell infusion

Before conditioning and cell infusion we will repeat or perform additional exams, tests, or procedures to make sure it is safe for you to receive the study treatment:

- Physical examination, including weight, height, and vital signs
- Review of your symptoms, medications, and your ability to perform your normal activities
- Routine blood and urine tests to test your immune function, organ function and to test for various infections (~10 tablespoons of blood)
- Blood or urine test if you are a woman or a girl who can have children
- Test to check if you were infected with tuberculosis
- Computed Tomography (CT) scan of chest, abdomen, and pelvis: The CT scanner is a doughnut-shaped machine that uses x-rays to create computer pictures showing the inside of your body. During the procedure, you will need to lie still on a table inside the CT machine. The table will move you in and out of the machine during the scan and you will be instructed to hold your breath.
- Dental exam to check for dental issues that need to be dealt with before transplant
- Eye exam
- Meeting with a dermatologist
- Meeting with a radiation oncologist
- Meeting with a social worker
- Meeting with a dietician to review your nutrition
- Meeting with an infectious disease specialist
- Meeting with a gynecologist (women and girls as appropriate)

Conditioning

You will start conditioning to help your bone marrow get ready to receive the transplant. The combination of medications and TBI you will receive is given to prepare you for the transplant so that your body will not reject the donor cells:

Group 1

- Briquilimab IV on any day between Days -13 and -10
- Fludarabine IV on Days -4, -3 and -2
- TBI on Day -1

Group 2

- Briquilimab IV on any day between Days -13 and -10
- Fludarabine IV on Days -6, -5, -4, -3 and -2
- Cyclophosphamide IV on Days -6 and -5
- TBI on Day -1

About 2 hours before the Briquilimab infusion, you will be given acetaminophen, diphenhydramine, and hydrocortisone to help prevent any side effects from this drug.

After the Briquilimab infusion, we will start to check the level of this drug in your blood because we want to infuse stem cells on the day when your Briquilimab drug level is predicted to be at the optimal level to allow for successful donor stem cell engraftment. We may adjust the day your chemotherapy conditioning starts, and Day 0 based on how quickly Briquilimab is cleared from your blood.

You will be hospitalized starting on the day prior to the start of the conditioning. Most people will remain hospitalized for about 4 weeks after the transplant, although this may vary depending on if there are complications.

During 2 weeks of conditioning, we will draw approximately 12 tablespoons of blood for routine tests to monitor your health.

Transplant

On the day of your transplant, you will receive the donor stem cells through the central venous catheter.

Four days before transplant and for approximately 30 days after transplant, you will be given ursodiol to help prevent any side effects from the cell infusion.

Immediately After Your Transplant

You will need to remain in the hospital for several weeks after your transplant. You will likely need transfusions of red blood cells and platelets during the first few weeks after your transplant until the donor stem cells start making red blood cells and platelets of their own. You may have a fever, throat pain, tiredness, lack of appetite, hair loss, skin changes, and/or other health issues that may arise during your transplant. You will be monitored closely for changes in your organ

function, blood counts, and signs of infection. You may need procedures, radiology scans, additional medications, or blood tests, apart from those routinely scheduled, if issues arise before, during, or after your transplant.

Medications to Prevent Graft Versus Host Disease (GVHD):

Starting on day 3 after your transplant, you will receive medicines to prevent GVHD. This medication will include a chemotherapy drug called cyclophosphamide. You will receive cyclophosphamide on day 3 and day 4 after the transplant. You will receive the medications tacrolimus and mycophenolate mofetil starting on day 5 after the transplant. You will continue mycophenolate mofetil for a few weeks after the transplant and tacrolimus for 6 months after the transplant. If you do not develop graft-versus-host disease, these medications will be stopped at designated time points after transplant. If you develop GVHD, you may need additional medications. A study doctor will discuss these medications with you if they become needed.

Medications to Prevent Infection

You will also take several standard of care medications to prevent infection after transplant. These are important to try to protect you from infections that can occur while your new immune system is getting stronger and learning how to recognize and fight infection. You may be given a daily medication after transplant until your white blood cell count comes up to near normal numbers.

Additional research testing

In addition to the tests that we will conduct to determine whether you are having side effects or if you are responding to the study therapy, we will also collect samples from you and perform tests for purposes of research only.

Blood samples will be collected:

- to study effects of the study therapy on your immune system before the start of conditioning, approximately 100, 180 days, 1 and 2 years after transplant (~ 1 tablespoon)
- to check the Briquilimab drug level in your blood immediately after Briquilimab infusion on Day -11, approximately 4, and 8 hours later, and then on Days -10, -9, -7, -3, -1 and 0 (~ 1 tablespoon)

When you are finished taking the drugs (treatment)

Once you no longer need frequent infusions of medications through the central venous catheter, transfusions, and nursing care and you are strong enough to carry out your daily activities on your own or with the help of your caregiver, you will be discharged from the hospital and required to remain in the Washington, D.C. area for approximately 100 days after transplant because you will be seen at least once a week in the NCI Oncology Clinic or day hospital. Each visit usually takes about 3 hours but may take up to 8 hours. You may require readmission to the hospital if there are complications.

From around day +100 on, most individuals will be allowed to return home. You will need to return for follow-up visits to the NIH around day 180 and yearly for 3 years. You will also need to have a doctor near your home who can follow you regularly once your visits at NIH become less frequent.

You will continue to need frequent blood draws and will likely require adjustments in your medications. Approximately 5 tablespoons of blood will be drawn for routine tests at each time point.

In the weeks after the transplant, we will also do blood tests to see how many of the white blood cells are coming from the donor immune system and how many are coming from your old immune system. This is one way that we measure the function of the donor stem cells in your body. If there are fewer donor stem cells than expected after transplant, you may need additional infusions of donor cells to improve the numbers. Bone marrow aspirations and biopsies may also be performed at about 100 days and 1 year after your transplant to evaluate the health of the bone marrow and the new immune system.

After your transplant, you will need to be re-vaccinated against infections. These vaccinations will start around 6 months after transplant, with delays possible depending on if your immune system is ready to handle the vaccines or not.

To reduce your chance of infection and other complications post-transplant, you will need to lessen your day-to-day household activities and public activities for at least 6 months after transplant. These restrictions could potentially extend for a longer time, depending on your immune system and organ function. Day-to-day activities include not cleaning bathrooms, dirty dishes, pet cages/aquariums/litter boxes, or dirty laundry, avoiding certain aspects of gardening, not mulching or mowing, not changing diapers of infants, not taking out the trash, and avoiding people who have received live vaccines (usually children) for 2 days after vaccination. It is recommended that you shower daily, brush your teeth daily, wash your hands frequently, and wear a mask in public for at least 3-4 months after transplant. This may include not returning to work or school for 6 months, avoiding large public events, avoiding active construction/dust, and not traveling to places without specialized medical care and resources. Also, you will be advised to not vape or smoke marijuana. You will be cautioned to not wear contact lenses for 3-4 months after transplant, as well as to avoid manicures, pedicures, and getting tattoos unless cleared to do so by your transplant team. You will be cautioned against sun exposure that leads to burning or significant tanning and you will be advised to wear sunblock. You will be advised to not get any new pets in the first 6 months after transplant and avoid caring for reptiles, amphibians, and birds during the first 6 months after transplant. You will be advised to not sleep with your pets.

HOW LONG WILL THE STUDY TAKE?

If you agree to take part in this study, you will be involved as described above, for 3 years.

HOW MANY PEOPLE WILL PARTICIPATE IN THIS STUDY?

Up to 32 people might get Briquilimab based conditioning and transplant on this study.

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

Risks of the transplant

Risk of death from transplant

In transplant studies that are in some ways similar to the current study's approach, the risk of death in the first year after transplant is around 10-20% (10 to 20 people out of 100 may die). The risk of death or other complications can vary greatly, depending on the age of the individual, the way

the transplant is performed, the health of the participant at the time of transplant, and other factors. There is also a risk of complications that cannot be predicted.

Risk of acquiring an allergy or other immune-system problem from a donor

Although highly unlikely, it is possible that you might acquire a serious problem with the immune system from a donor. All donors are screened for signs of immunodeficiency. It is possible to acquire allergies, such as drugs, food, or environmental allergies from your donor. Donors are asked about serious or significant allergies, but they may not be aware of all of their allergies. This is particularly true for drug allergies, as donors are often exposed to very few drugs by nature of their good health. If a donor does have a significant drug allergy, we generally avoid giving you that family of drugs after transplant. It is also possible that other immune system problems could be acquired from your donor, such as autoimmune problems. We screen donors by asking them extensive questions about significant autoimmune problems and do not use donors who have known autoimmune problems that interfere with their health.

Risk of graft rejection

In the first 2-4 weeks after transplant, nearly all participants will have very low blood counts. Engraftment – when the donor's stem cells start making blood cells for you – usually occurs between days +12 and +28 after transplant. There is a small chance (5 to 10 people out of 100 may have this) that you may reject your donor's stem cells. If that were to happen, your own blood cells would likely recover, and you would then decide whether to receive standard therapy or proceed with another transplant. However, there is a possibility your own blood cells may not recover, and you would need another transplant either from the same or a different donor in an urgent fashion to decrease your risk of infection, bleeding, and death. During the time before the second transplant, if needed, we would attempt to support you with transfusions, growth factors, and antibiotics.

Risk of infertility

This treatment may result in the inability to have children in the future. This is true for any type of transplant and many other therapies in which chemotherapy and/or radiation are used. These types of therapies are used as they also decrease the chance of the primary immunodeficiency disease returning. 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. If you are interested in being evaluated by a reproductive endocrinologist to discuss fertility preservation prior to the transplant, we can assist you in starting this process. If you are a man and interested in fathering a child in the future, you may want to consider sperm banking prior to transplant.

Risk of stem cell infusion

The donor stem cells will be infused through your central venous catheter and will appear very similar to a blood transfusion. A small number of people (less than 5 people out of 100) may develop a fever, chills, body aches, trouble breathing, anemia, or dark urine during or after the stem cell infusion. This does not mean that your body is rejecting the graft (donor cells). These side effects usually occur because of other cells and proteins, apart from the stem cells, that react with your body. The infusion may need to be slowed down or held for a short time but will ultimately be infused completely. It would only be in the extremely rare circumstance where the

infusion is deemed medically unsafe to continue that the stem cell infusion would not be completed, although this is not anticipated to occur.

Risk of nutritional decline

Going through a transplant is a time of high calorie and high protein needs for your body. Despite these high needs, you may feel unwell and may be unable to keep up with the high energy demands on your body. This can lead to weight loss, muscle mass loss, and nutritional deficiency. These issues are usually temporary. You will receive an evaluation by a dietician throughout your transplant course to assist in finding ways to meet your body's energy demands during the transplant and minimize the risk of nutritional decline.

Risk of infection

The most common complication after transplant is infection. This includes infections from viruses, bacteria, fungus, and other sources. If you have active infections at the time of transplant, there is a risk that these infections may worsen, particularly in the early days of transplant. Even though you are receiving a transplant to improve your blood and immune system, this improvement does not happen immediately, even if the transplant is completely successful. Even without any complications, the new immune system will take months to years after transplant to work normally. You will also be receiving chemotherapy and medications to prevent graft-versus-host disease that will decrease your immune system's ability to fight infection. You will be monitored closely for infection and treated if there are effective therapies. There are some infections that can occur after transplant that lack effective therapies.

Risk of a sinusoidal obstructive syndrome (SOS)

A severe liver complication known as SOS occurs in about 5 people out of 100 of allogeneic blood or bone marrow transplants. SOS is a chemotherapy side effect that causes the blood vessels of the liver to be blocked. The risk of SOS in this study is expected to be around 5%. Cyclophosphamide treatment may increase the chance of having SOS. Other factors such as prior liver disease may increase the risk of SOS. Severe SOS can lead to liver failure and death.

Risk of other organ toxicity

Complications of other organs besides the liver can occur after transplant, particularly the lungs. Lung complications can be infectious but also can result from damage from chemotherapy and/or inflammation related to the donor immune system operating in the recipient's body. Although these lung complications are infrequent (occurring in 3-8 out of 100 people), they can rarely be severe or life-threatening and can sometimes result in irreversible lung damage.

Risk of Graft-versus-Host Disease (GVHD)

You will be at risk for the development of GVHD for the rest of your life after transplant, although this problem, when it occurs, almost always begins within the first year or two after transplant. There are two forms of GVHD – one that usually occurs earlier after transplant called "acute GVHD" and one that usually occurs later after transplant called "chronic GVHD". Acute GVHD most commonly attacks the skin, liver, and/or gut. Symptoms of skin GVHD may be as mild as an itchy rash or as severe as blistering and loss of the skin. Symptoms of gut acute GVHD may be as mild as heartburn, nausea, or mild diarrhea, or as severe as cramping abdominal pain and large volumes of diarrhea. Liver acute GVHD may be as mild as a slight disturbance in liver function,

or as severe as jaundice (yellowing of the skin) with liver failure. Mild acute GVHD can be treated with steroid creams applied to your skin. Severe GVHD can be very dangerous and needs to be treated aggressively. Treatment of severe acute GVHD included weakening the immune system with steroids and other medications. This can increase your risk of infection, cancer, and death. Chronic GVHD, if it occurs, typically appears later after transplant, usually starting between 3-12 months after transplant. Some degree of chronic GVHD affects about half of individuals after conventional transplant. With the use of post-transplantation cyclophosphamide, the risk of chronic GVHD is much lower at around 10-15% (10 to 15 people out of 100 may develop it). Chronic GVHD commonly attacks the skin, eyes, mouth, liver, or intestines, but can also involve the lungs, muscles, joints, bone marrow, and other organs. Symptoms of chronic GVHD may include dryness of the mouth or eyes, loss of appetite, weakness, hair loss, changes in skin color or texture, liver damage, weight loss, shortness of breath, cough, or other symptoms. People with severe chronic GVHD are also at increased risk of infection or death. Chronic GVHD is also treated with drugs to weaken the immune system, such as steroids. Taking steroids increases your risk of infection, bone thinning, diabetes, cataracts, weight gain, and other complications.

Risk of late transplant complications

There are other potential complications that can occur long after a transplant. These could affect any organ in the body and are usually due to late effects of damage done by chemotherapy or other medications used for transplant. People who get transplants are at increased risk for developing certain types of cancers such as cancers of the mouth, throat, and skin. Some of that increased cancer risk is related to chronic graft-versus-host disease, which we hope to avoid, but some are related to prior chemotherapy exposure.

DMSO Risk

Cells you are going to receive may be frozen and stored with a preservative called DMSO. Side effects may include nausea, vomiting, and diarrhea. Most commonly it causes an unpleasant taste and smell (like garlic). Other side effects that have been reported include facial flushing, loss of appetite, and flu-like symptoms.

Risks of Study Drugs

The study drugs used in this study may affect how different parts of your body work such as your liver, kidneys, heart, and blood. The study doctor will be testing your blood and will let you know if changes occur that may affect your health.

There is also a risk that you could have side effects from the study drug(s)/study approach.

Here are important points about side effects:

- The study doctors do not know who will or will not have side effects.
- Some side effects may go away soon, some may last a long time, or some may never go away.
- Some side effects may interfere with your ability to have children.
- Some side effects may be serious and may even result in death.

Here are important points about how you and the study doctor can make side effects less of a problem:

- Tell the study doctor if you notice or feel anything different so they can see if you are having a side effect.
- The study doctor may be able to treat some side effects.

The study doctor may adjust the study drugs to try to reduce side effects

Briquilimab

Likely

- Headache
- Low hemoglobin
- Cough
- Itching throat
- Nasal congestion
- Fever
- Rash
- Nausea
- Vomiting
- Low blood counts
- Bone marrow making less red blood cells
- Bleeding from the nose

Less likely

- Tingling or prickling, “pins-and-needles” sensation in arms and legs
- Severe hypersensitivity

Fludarabine

Likely	Less Likely	Rare but Serious
• Low blood counts	• Nausea, vomiting • Long term reduction of lymphocyte counts which could increase the risk of infection • Infection	• Seizures, coma, blindness, other neurologic toxicity, and even death • Inflammation in the lungs • Kidney damage • Allergic reaction

Cyclophosphamide

COMMON, SOME MAY BE SERIOUS
• Fever • Infection, especially when white blood cell count is low • Anemia which may cause tiredness, or may require transfusion • Bruising, bleeding • Blood in urine • Nausea, vomiting, diarrhea, loss of appetite, pain in belly • Sores in mouth which may cause difficulty swallowing • Absence of menstrual period which may decrease the ability to have children • Hair loss, skin changes, rash, change in nails • Blurred vision, vision changes

OCCASIONAL, SOME MAY BE SERIOUS
• Fluid around the heart • Damage to the bone marrow (irreversible) which may cause infection, bleeding, may require transfusions • Loss or absence of sperm which may lead to an inability to father children

RARE, AND SERIOUS
• Damage to the heart or heart failure which may cause shortness of breath, swelling of ankles, cough or tiredness • Swelling of the body including the brain which may cause dizziness, confusion • Damage to the lungs or scarring of the lungs which may cause shortness of breath • Allergic reaction which may cause rash, low blood pressure, wheezing, shortness of breath, swelling of the face or throat • Hepatic veno-occlusive disease is a condition that is characterized by damage to blood vessels in the liver and liver cells. Although it may be mild and not require further treatment, sometimes it may cause a severe decrease in liver function and may be life threatening or fatal. • Kidney damage which may cause swelling, may require dialysis • A new cancer (e.g., leukemia, lymphoma, sarcoma etc.) resulting from treatment of a prior cancer • Severe skin rash with blisters and peeling which can involve mouth and other parts of the body. • Impaired wound healing • Urinary and/ or kidney including blood in urine, painful urination, fever, urgency, inability to urinate, loss of bladder control and pain.

RARE, AND SERIOUS
• Abnormal heartbeats: including atrial fibrillation and flutter and ventricular arrhythmias causing your heart to be fast or irregular resulting in a pounding or racing heart, dizziness, weakness, feeling light-headed or shortness of breath. • Decreased levels of sodium in the blood, which can cause confusion, seizures, fatigue and low levels of consciousness.

In addition, because cyclophosphamide may contain alcohol, it may impair a person's ability to drive or operate machinery immediately after the infusion.

Mycophenolate Mofetil

Likely	Less Likely	Rare but Serious
• Neutropenia (decrease in white blood cells) • Anemia • Indigestion and/or nausea • Diarrhea • Risk of infection	• Acne • Rash • Itching	• Secondary cancers • Allergic reaction

Tacrolimus

Likely:	Less likely:	Rare:
• headache • tremor • changes in mental status • high blood pressure • abnormal kidney function • constipation, diarrhea • abdominal pain • difficulty sleeping (insomnia)	• liver inflammation • diabetes • anemia • an allergic reaction • sensitivity reaction to light • elevated lipid (fat) levels in the blood	• seizure • coma

Fludarabine and cyclophosphamide will reduce cell counts that increase your chance of infection. These infections can be very serious and may result in death. If you develop signs of an infection, you may need additional blood tests, scans, medications, and other treatments.

These medications will also reduce your counts for other blood cells. This will increase your chance of bleeding or bruising, and you will feel a lack of energy or other issues. A blood transfusion is often given to treat these issues.

Risks of Study Procedures

Blood collection

Side effects of blood draw include pain and bruising in the area where the needle was placed, lightheadedness, and rarely, fainting and infection. When large amounts of blood are collected, a low red blood cell count (anemia) can develop. We will not collect more than 15 tablespoons of blood during any day on study or 44 ½ tablespoons within 8-week period.

Central Venous Catheter (CVC)

Risks include pain, bleeding, infection, bruising, blood clot, or infection at the site where the catheter is put in. In rare cases, placing a CVC has resulted in the collapse of a lung. If this happens, the lung would be quickly re-inflated using a tube put into your chest. Sometimes catheters may become infected or clogged. If this happens the catheter may need to be replaced. The CVC will be flushed once a day to prevent it from becoming clogged. The nursing staff will show you how to do this yourself when you return home.

Bone Marrow Aspiration / Biopsy

This procedure usually causes only brief discomfort at the site from which the biopsy is taken. Risks of biopsy may include bleeding, bruising, injury to internal organs, and infection. Serious risks are very rare but include fat embolism (fat from the bone marrow enters the blood and goes to another part of the body, blocking the blood flow). Rarely, these complications from biopsy could result in hospitalization and require additional medical care.

Sedation

A bone marrow aspiration/biopsy and CVC placement may be performed under conscious sedation with Fentanyl and Midazolam (Versed). The most common risks of sedation last up to a few hours after being given and can include drowsiness, feeling slow or sluggish, low blood pressure, headache, and nausea.

ANESTHESIA

The risks of sedation include decreased rate of breathing while under sedation and aspiration (saliva or stomach contents breathed into the lungs). Additional risks include a drop in heart rate or blood pressure. In the rare event that this should occur, the anesthesiologist may have to put a longer breathing tube into your mouth and windpipe, use a respirator to breathe for you, and give medications to raise your blood pressure. If you have a severe reaction during the sedation procedure you will be resuscitated regardless of whether or not you have made plans not to be resuscitated under normal circumstances. Please inform us if you or a family member has had problems with sedation or anesthesia in the past.

ANESTHESIA IN PEDIATRICS

The FDA has issued a safety warning about anesthesia in children, especially anesthesia lasting longer than three hours or repeated anesthesia even if it is brief. Research has shown learning and behavioral problems in animals undergoing long anesthesia or repeated brief anesthesia. Similar problems may be more likely in children who have had long anesthesia or repeated brief anesthesia. However, research in children has not found learning or behavior problems after one

short exposure to anesthesia. An anesthesiologist will talk to you about the risks and benefits of general anesthesia being used in this study for your child.

CT scans

If contrast dye is used, there is a risk for allergic reaction to the dye. Participants might experience hives, itching, headache, difficulty breathing, increased heart rate, and swelling. If you are allergic to or sensitive to medications, contrast dye, iodine, or shellfish, please notify your study doctor. If you have had kidney failure or other kidney problems in the past, please notify your study doctor.

Electrocardiogram

You may experience some minor skin irritation from the electrodes.

Echocardiogram of ECHO and urine collection

There are no known physical risks from these procedures.

Pulmonary (lung) function testing

These tests measure how well your lungs work. They are usually safe for most people. However, because the test may require you to breathe in and out quickly, you may feel dizzy. There's a small risk that you might faint. If you have asthma, this test could cause you to have an asthma attack. In very rare cases, pulmonary function tests may cause a collapsed lung. If you have asthma or feel lightheaded during the test, tell your doctor.

Privacy Risks Associated with Genetic Testing

It may be possible that genetic information from you could be used by law enforcement agencies or other entities to identify you or your blood relatives.

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 will need to practice an effective form of birth control before starting study treatment, and for a year after transplant or twelve (12) months after you finish conditioning (the restricted period for women). The conditioning medications can cause harm to a fetus, including birth defects, delayed growth, and miscarriage. 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 during the restricted period. If you think or know you have become pregnant during the restricted period, please contact the study team as soon as possible.

You must use effective birth control methods and try not to become pregnant or father a child before study treatment and for 1 year after transplant. If the transplant is not done, you must avoid making someone pregnant for 4 months after you finish chemotherapy if you are male. If you are female and the transplant is not done, you should avoid becoming pregnant for 12 months after you finish chemotherapy. If you think or know you have become pregnant or fathered a child during that period, please contact the study team as soon as possible. There may be unknown risks to the fetus or risks we did not anticipate. You and your partner must agree to use birth control if you want to take part in this study. If you think your partner has become pregnant during the restricted period, please contact the study team as soon as possible. If you and your partner plan for your partner to become pregnant after the restricted period, please discuss this with the study team.

Please, use effective birth control method from the list below:

- intrauterine device (IUD)
- hormonal (birth control pills, injections, or implants)
- tubal ligation
- vasectomy

What are the risks of radiation from being in the study

During a year in this research study, your bone marrow will be exposed to 200 cGY of radiation from the TBI used to clear your bone marrow. You will also receive a much smaller amount of radiation from scans used to plan your treatment and measure your progress. The total scans include one CT scan. The amount of radiation from these scans adds minimal additional risk to the higher radiation doses received in the course of treatment. This radiation has been reviewed by the NIH Radiation Safety Committee and deemed appropriate for this study.

Risks of Total Body Irradiation

During or shortly after treatment
Common: • Fever • Hair loss • Nausea and vomiting • Diarrhea • Mucositis (irritation at the lining of the mouth, throat, intestines) causes pain • Decreased blood cell count leading to infection or bleeding • Minor skin irritation • Mouth dryness • Parotiditis (irritation of the major salivary glands)
Uncommon: • Damage to lung, liver, and kidneys

After Treatment (months to years)
Common: • Sterility • Menopause • Cataracts • Reduced school performance in children (age 7 or younger)
Uncommon: • Thyroid gland doesn't produce enough thyroid hormone • Small blood vessels that lead into the liver and are inside the liver become blocked • Persistent shortness of breath • Permanent damage to liver, lung, and kidneys
Rare: • Other tumors • Death (extremely rare)

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 an improvement of your disease.

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

In the future, other people might benefit from this study because of the knowledge that will be gained.

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:

- get treated with medicines that are already approved by the FDA for your illness
- join a different study if there is one
- decide not to get treated for GATA2 deficiency, but you might want comfort care to ease the symptoms

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

The results of the standard tests performed as part of the research are available to you as part of your medical record.

EARLY WITHDRAWAL FROM THE STUDY

Your doctor may decide to stop your therapy for the following reasons:

- if your disease worsens during treatment
- if you have strong side effects from the study drugs
- if transplant did not work
- donor is unavailable
- if you do not get transplant for any reason
- if you become pregnant
- if the study is stopped for any reason

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

If you received a transplant, we would like to see you for a safety visit 100 days after the transplant. If you did not receive a transplant, but started study therapy, we would like to see you for a safety visit 30 days after you last received Briquilimab.

You can choose to stop being part in the study at any time. But if you decide to stop, we would like you to talk to the study doctor and your regular doctor first.

If you decide to withdraw your consent to be part of the trial, we will not collect any more medical information about you. However, information we collect about you up to that point may still be provided to Jasper Therapeutics or designated representatives following FDA guidelines.

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 GATA2 deficiency, 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

In some cases, it may help other researchers to know that the specimens or data were collected from you (i.e., they will have your identifiers). If we share your identity with other researchers, their study will be reviewed and approved by an Institutional Review Board who will make sure that the study team is protecting your confidentiality.

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

_____ Yes _____ No

Initial Initial

May we use your specimens or data for genetic research?

We may also save your samples and use them for genetic research in the future, possibly as part of a different study. Genetic research involves studying your DNA or RNA. Your genes are made up of DNA, short for deoxyribonucleic acid. DNA contains information that determines in part the traits, such as eye color, height, or disease risk, that are passed on from parent to child. RNA is short for ribonucleic acid. RNA is a genetic material, made following the instructions from DNA, that has a major role in making proteins. Proteins are the building blocks of your body, cells, and organs. We may do genetic research to identify genes that cause or contribute to a disease or trait.

I give permission for my specimens and data to be used for genetic testing in 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 that 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 indefinitely.

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?

On this study, the NCI will reimburse the cost for some of your expenses such as those for hotel, travel, meals. Some of these costs may be paid directly by the NIH and some may be reimbursed after you have paid. The amount and form of these payments are determined by the NCI Travel and Lodging Reimbursement Policy. You will be given a summary of the policy which provides more information.

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.

- 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 generally 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.

CONFLICT OF INTEREST (COI)

The National Institutes of Health (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 lentiviral vector developed by Jasper Therapeutics through a joint study with your 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, Center for Cancer Research of National Cancer Institute.
- Qualified representatives from Jasper Therapeutics, the pharmaceutical companies who provides Briquilimab.

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.

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, Danielle E. Pregent-Arnold, M.D., Telephone: 240-281-3922, Email: danielle.arnold@nih.gov. 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

Assent: I have had this study explained to me in a way that I understand, I have been given the opportunity to discuss it, and I have had the chance to ask questions. I agree to take part in this study.

Assent of Minor:

Signature of Minor	Print Name of Minor	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: _____.