

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:** National Institutes of Health Clinical Center**Cohort:** Assent (12-17 year olds)**Assent Version:** 01/22/2025

What is a research study?

Research studies help us learn new things. We can test new ideas. First, we ask a question. Then we try to find the answer.

This paper talks about a research study that we are doing and the choice that you have to take part in it. You are being asked to join this research study because you have the disease of the immune system called GATA2 deficiency. We want you to ask us any questions that you have. You can ask questions any time.

Important things to know...

- You get to decide if you want to take part.
- You can say ‘No’, or you can say ‘Yes’.
- No one will be mad at you if you say ‘No’.



Why are we doing this research?

We are doing this research to look at a new therapy in children with the disease of the immune system called GATA2 deficiency. We think your disease may be improved and perhaps even cured with a transplant.

A transplant is a procedure where blood “stem cells” from another person are given to you through a plastic tube in the veins. These “stem cells” may grow into all the different cells that make up your immune system.

To prepare your immune system to accept transplant, we will use a new drug Briquilimab (also called JSP191) in combination with other drugs and total body irradiation (TBI).

This is investigational therapy. “Investigational” means that doctors are doing more research to learn more about it. The main purpose of the study is to see if a blood transplant from another person in combination with a new drug Briquilimab will work.

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The study doctor would like to find out if you can be on this research study. To find out if you can be on the study, we first need to get some information about you. Some tests will need to be done to make sure you can join the research study if you choose to. This is called screening.



What would happen if I screen for this study?

If you decide to be screened for the study, we will ask you to do the following:

- Medical records: We will look at your past doctor visits and use information about your care
- Medical history: We will ask questions about your health, current medications, and any allergies
- Physical exam: A doctor will look at your skin, listen to your heart, lungs, and stomach. You will also have your height, weight, temperature, blood pressure and how much oxygen you have in your blood checked
- Performance status, which evaluates how you are able to carry on with your usual activities.
- Blood tests: You may need a needle poke so we could test some of your blood. If possible, we will try to get blood without a new poke
- Other tests (to check your urine, liver, possibly a pregnancy test)
- Tests to check your heart and possibly lungs
- Bone marrow aspiration/biopsy: Bone marrow aspiration is when we take a sample of the fluid from your hip with a needle. A bone marrow biopsy is the removal of a small amount of solid tissue using a needle

If you are able to join and want to be in this study, we will ask you to sign this form.



What would happen if I join this research?

Before receiving the transplant, you will be given drugs (Briquilimab and fludarabine) and have a procedure called total body irradiation (TBI) to help prepare your immune system to accept the transplant (preparative procedures). Depending on the donor, you also may be given an additional drug, cyclophosphamide. Fludarabine and cyclophosphamide are chemotherapy drugs that are approved for use by the FDA and are not investigational. All drugs will be given to you as an intravenous (IV) infusion, which means by a tube in one of your veins. A few weeks after the transplant, doctors will do some tests to see if the transplant worked. From the time that you start preparative procedures, you will be in the hospital until the doctors feel that it is safe for you to stay outside the hospital.

After the transplant, we will give you drugs to prevent graft-versus-host disease (GVHD), a condition in which the donor cells attack your own normal cells.

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Preparative procedures and a transplant will make you feel sick, and you will stay in the hospital until you feel better. When we let you go home, we will ask you to stay close to NIH (i.e., at the Children's Inn) until Day 100, so the doctors and nurses keep watching to make sure that you are ok.

Throughout the study, to see how your disease is doing, you will come to the clinic to get some of the same tests repeated as you did to see whether you could join the study. Some of these tests include:

- Doctor's visits, and physical exams
- Tests to see how your heart work
- Blood tests: You may need a needle poke so we could test some of your blood. If possible, we will try to get blood without a new poke
- Other tests (to check your urine, liver, possibly a pregnancy test)
- Imaging with CT scan. The CT scanner is a doughnut-shaped machine that uses x-rays to create computer pictures showing the inside of your body. 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 told to hold your breath. The scan itself will only take a few minutes to complete, the entire visit will take about 30 minutes.
- Bone marrow aspiration/biopsy: A sample of the fluid from your hip with a needle. A bone marrow biopsy is the removal of a small amount of solid tissue using a needle. You may be put to sleep (general anesthesia) for the procedure so that you are not uncomfortable, or you may be given medicine to help you relax and to prevent pain.

You will participate in this study for 3 years.



Could bad things happen if I join this research study?

Some of the tests or procedures might be uncomfortable. Doctors and nurses will try to reduce your discomfort and help explain what may happen and how you may feel. It is very important to tell your parents/guardians and doctors if you feel sick or have pain anywhere in your body. We will try to make sure that no bad things happen.

You can say 'no' to what we ask you to do for the research at any time and we will stop.

These study drugs and a transplant can make you very sick. You may have nausea, vomiting, feel tired, dizzy, have trouble sleeping, headache, earaches, jaw pain, muscle weakness, constipation, skin rash or peeling, nose bleeds, and diarrhea. We will also give you other medicines to protect you from the side effects of the study drugs.

This type of treatment can lower the number of different blood cells in your body. This can be serious because blood cells do a lot in your body: fight off infections, carry oxygen, and prevent bleeding. If you feel bad or have a fever during this treatment it is important to tell your parents or doctor right away, so that they can help you.

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Some of the things might happen to you or they might not. Or things might happen that we don't know about yet.

For a few months after your transplant, your immune system will be weak, and you need to be very careful, try to regularly wash your hands and use clean things around you. You will not go to school or places with a lot of people or play with other children.

You may have some, all, or none of the following, as part of the tests done to see if you are able to be a part of the study:

- The poke to test your blood can hurt. Sometimes the needle can leave a bruise on the skin. We can put a cream on your skin before we take blood. This cream can help so it won't hurt as much
- Discomfort from small stickers that are attached to wires that go to the machine on the chest and arms/legs from tests to see how your heart is working
- Pain, bruising and bleeding, and sometimes an infection from the bone marrow biopsy, or apheresis needles
- You may be put to sleep (general anesthesia) for the procedure so that you are not uncomfortable, or you may be given medicine to help you relax and to prevent pain. You may feel sleepy from the medicine for a few hours, or have a headache.
- The chemotherapy given to prepare your body for the transplant may make you feel sick or nauseous or you may be allergic to it
- CT Scan (computerized tomography): CT scans expose you to radiation. You could have an allergic reaction to the dye (contrast) that is used to make the images clear, have a rash or trouble breathing, and feel uncomfortable when the contrast is injected
- Lung function testing: These tests 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 or have sudden difficulty breathing. If you have asthma, this test could cause you to have an asthma attack

Due to risks to an unborn fetus or baby, you must avoid getting pregnant or making someone pregnant 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 a girl and the transplant is not done you should avoid becoming pregnant for 12 months after you finish chemotherapy.

Your medical team will talk to you about all of this and any concerns that you have. They will continue to tell you about what will happen or what is happening. If you have any concerns, you should always talk to your care team.

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Could the research help me?

We think being in this research may help you because you may have better blood cell counts, fewer infections, and/or cure of your bone marrow abnormalities. We hope that information we learn will benefit people with GATA2 deficiency in the future.



What else should I know about the research?

Please talk to your parents about this before you decide whether or not to be in this research study. We will also ask your parents to give their permission for you to be in this study. But even if your parents say “yes,” you can still decide not to be in this research study.

If you don’t want to be in this study, you don’t have to.

Remember, being in this study is up to you and no one will be upset if you don’t want to take part in this study.

It is also OK to say ‘yes’ and change your mind later. You can stop being in the research at any time. If you want to stop, please tell the research doctors. If you do stop, we may ask you to come back to see us at least one more time to see how you are doing.

You will not receive anything for being in the study.

Once you have turned 18, we will contact you to find out if you would still like to participate in the study.



What if I have any questions?

You can ask questions any time. You can talk to me, Dr. Pregent-Arnold, at 240-281-3922. Ask us any questions you have. Take the time you need to make your choice.

**Is there anything else?**

If you want to be in the research study after we talk, please write your name below. We will write our name too. This shows we talked about the research and that you want to take part.

Assent of Minor:

Signature of Minor

Print Name of Minor

Date**Investigator:**

Signature of Investigator

Print Name of Investigator

Date**PATIENT IDENTIFICATION****Assent to Participate in a Clinical Research Study**

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