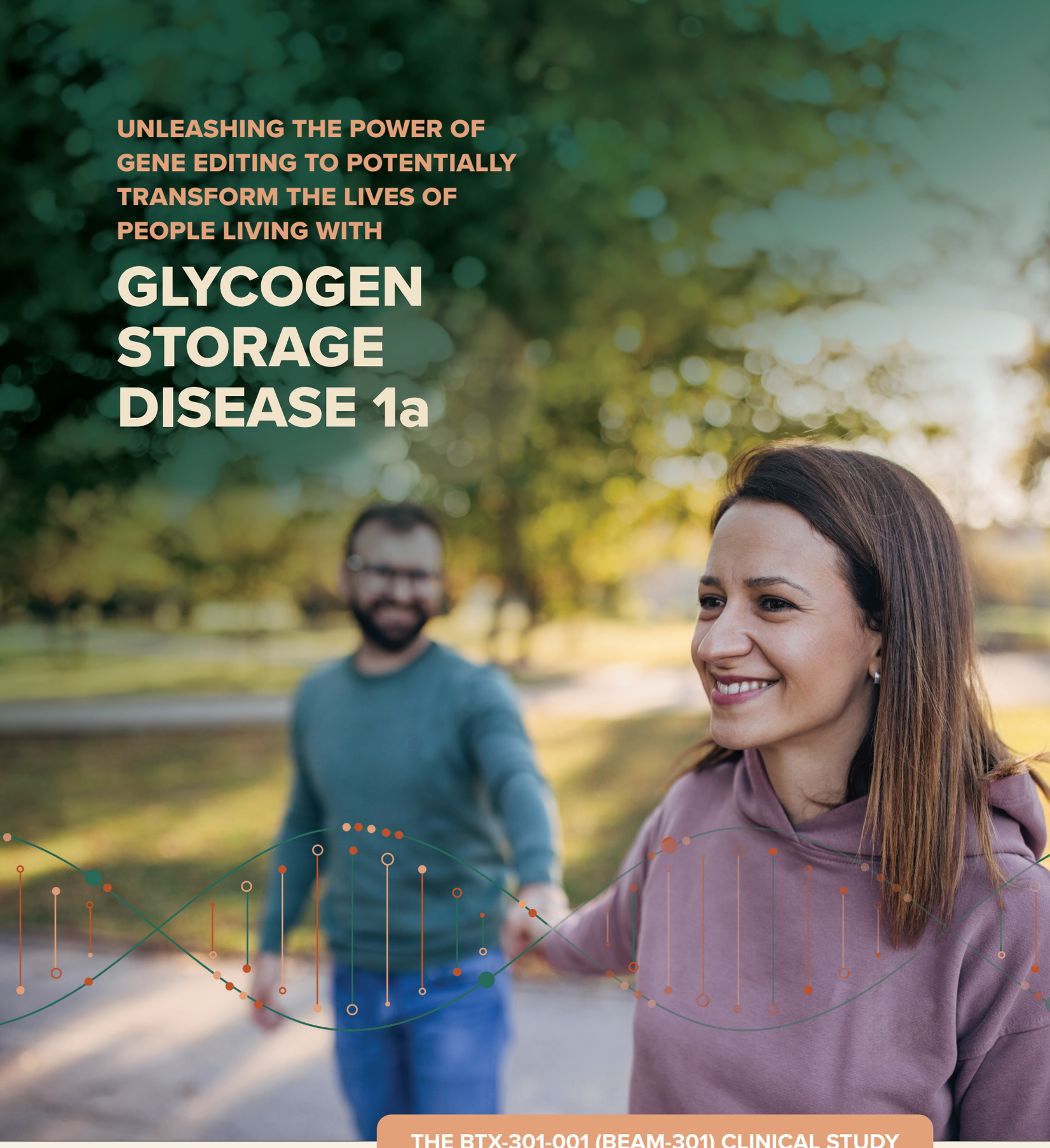


UNLEASHING THE POWER OF GENE EDITING TO POTENTIALLY TRANSFORM THE LIVES OF PEOPLE LIVING WITH

GLYCOGEN STORAGE DISEASE 1a

THE BTX-301-001 (BEAM-301) CLINICAL STUDY

The **BEAM-301 clinical study** is for individuals who have GSD1a with at least one copy of the R83C variant.

This brochure is your guide to the BEAM-301 clinical study.

THANK YOU FOR YOUR INTEREST IN THE BEAM-301 CLINICAL STUDY

With the help of potential study participants like you, new and improved therapies—and even a possible cure—for GSD1a may be discovered and help transform patients' lives.

Please consider joining us!

WHO WE ARE

We are Beam Therapeutics Inc, founded in 2017 to develop advanced precision genetic medicines. Our vision is to provide lifelong cures for people suffering from serious diseases. To achieve that, we have built a strong, values-driven organization focused on people, advancing cutting-edge science, and developing a new class of genetic medicines for patients who have unmet needs.

Beam THERAPEUTICS

WHAT IS A CLINICAL STUDY?

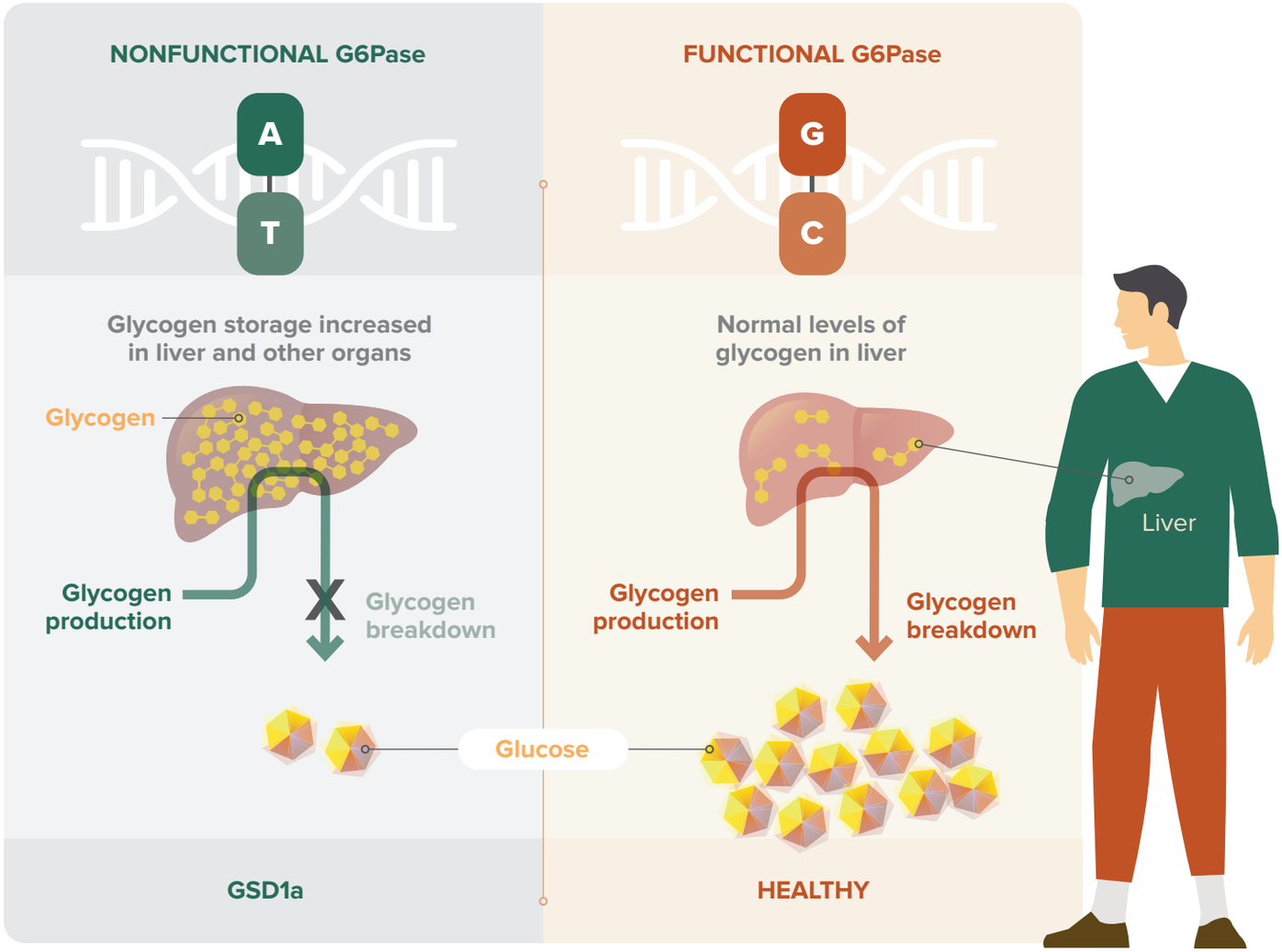
A clinical study, also known as a clinical trial, is a type of medical research. A clinical study is conducted among people who *volunteer* to take part in the study. People who want to participate will sign a consent form after having the opportunity to review the study requirements and ask questions of the study team, including the study doctor. The research helps doctors and the US Food and Drug Administration (FDA) learn whether a potential treatment works and is safe.

With the BEAM-301 Clinical Study, a potentially new therapy may be possible for people who have GSD1a.

GSD1a KEY FACTS

Glycogen storage disease type 1a (also known as GSDIa or GSD1a) is a genetic disorder that affects how glycogen, a complex sugar, is converted into glucose for use as an energy source by the body.

- GSD1a is caused by having two changes, or mutations, one from each parent, in the *G6PC* gene. The *G6PC* gene produces an enzyme primarily in the liver called G6Pase. Mutations in the *G6PC* gene result in varying levels of G6Pase deficiency
- Glucose is a type of sugar that provides the body with energy. Unused glucose is stored in the liver (and other tissues) as glycogen. When the body needs glucose for energy, G6Pase helps release glucose from its storage form, glycogen
- People who don't have enough G6Pase cannot release glucose from glycogen, putting them at risk of severe, life-threatening low blood sugar (hypoglycemia) and requiring them to frequently consume complex starches
- Excess glycogen accumulates in the liver, kidney, and other organs, as it cannot be released properly. In addition, people who have GSD1a must severely restrict their diet to prevent the conversion of dietary glucose into excess triglycerides, cholesterol, and lactate, which can cause organ dysfunction and negatively impact their health



A GENE VARIANT CALLED R83C IS ONE OF THE MORE COMMON GENETIC CHANGES ASSOCIATED WITH GSD1a.

THE BEAM-301 CLINICAL STUDY

Clinical studies offer opportunities for study participants to partner with researchers to help develop potentially new therapies for diseases.

Purpose

The BEAM-301 Clinical Study is a phase 1/2 study that will evaluate the safety and efficacy (how well the potential treatment works) of BEAM-301 in people who have GSD1a and at least one copy of the R83C variant.

Goals

The goals of the BEAM-301 Clinical Study are to learn about the safety and potential side effects of BEAM-301 and whether BEAM -301

- decreases the symptoms of GSD1a, including hypoglycemia and high lactate levels
- enables a reduction or elimination of starch supplementation
- improves the quality of life and ability to function among those living with the condition

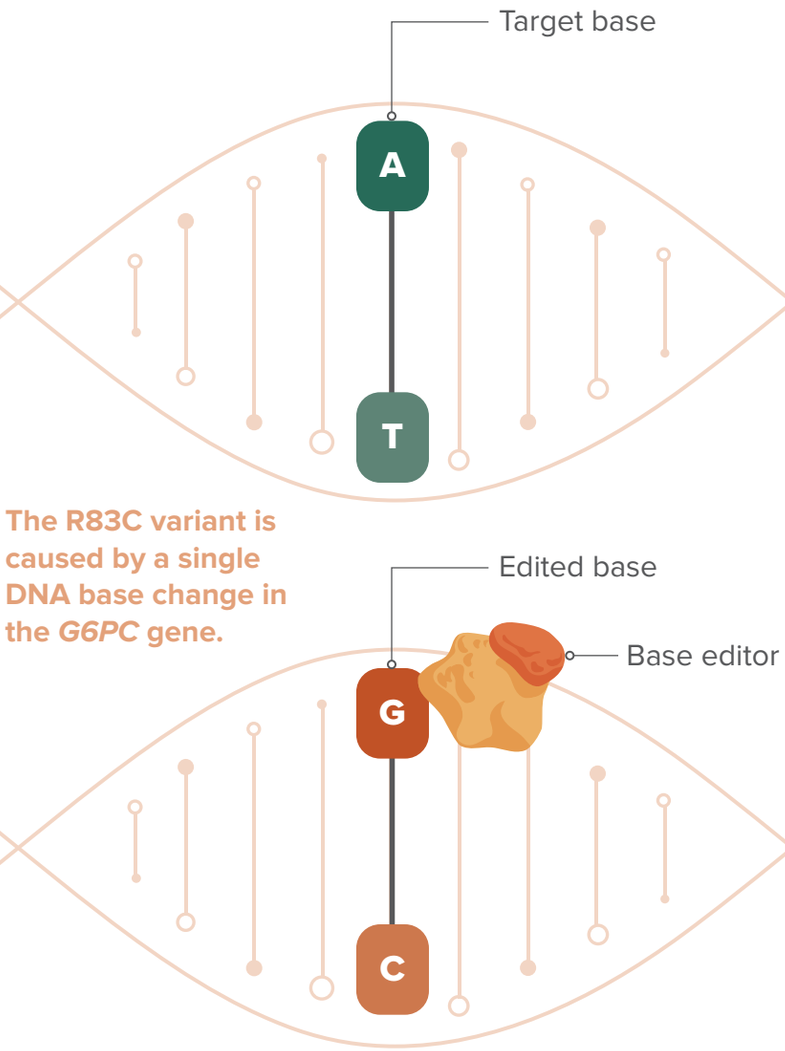
ABOUT BASE EDITING AND BEAM-301

What is a DNA base?

The foundational unit of your genetic information is a single DNA base. These bases pair up in a specific way and form sequences that spell out the genetic information carried in your DNA, much like letters of the alphabet are sequenced to form the words in a language. A change to a single base can mean the difference between health and disease.

What are base editing therapies?

Base-editing therapies are an emerging new class of precision genetic medicines designed to overcome the limitations of existing therapies. Base editors can edit a single base in DNA. That very basic edit can potentially treat a wide range of conditions.



The R83C variant is caused by a single DNA base change in the *G6PC* gene.

How does Beam-301* work?

Through base editing, BEAM-301 is designed to correct the R83C variant in the *G6PC* gene associated with GSD1a.

- BEAM-301 consists of base editing machinery encapsulated by lipid nanoparticles, which are composed of fats
- BEAM-301 is administered through an intravenous infusion and travels to the liver
- If the gene is corrected, the liver will produce G6Pase, the enzyme deficient in GSD1a

*BEAM-301 is an investigational drug because it is not approved by the US Food and Drug administration, European Medicines Agency, or any other regulatory authority.

TAKING PART IN THE BEAM-301 CLINICAL STUDY

How is the BEAM-301 Clinical Study being conducted?

BEAM-301 therapy will be studied

- in up to 36 adult participants
- at approximately 3 study sites in the United States
- in approximately 18 visits over approximately 2 years with short hospital stays for controlled fasting challenges and dosing
- in an additional long-term follow-up (LTFU) study, with minimal participant requirements, to provide insights into any possible long-term side effects of BEAM-301

Do I qualify to participate?

Adults aged 18 years or older who have a confirmed genetic diagnosis of GSD1a with one or two copies of the R83C variant may be eligible to participate. Participation is voluntary and can be stopped at any time.

In addition, eligible patients must

- have had at least 1 episode of hypoglycemia (<60 mg/dL) within 2 years before providing consent
- have been taking starch supplementation at least every 8 hours during the daytime hours to prevent hypoglycemia
- be willing to use highly effective contraception
- be willing to participate in this study (about 2 years) and an additional LTFU study (about 13 years)
- not have received a liver transplant or be waiting for one
- not have diabetes
- not have a liver adenoma larger than 5 cm or one between 3 and 5 cm with a documented annual growth rate of ≥0.5 cm/year
- not have a serious illness, infectious disease, or medical condition, including cancer, that could impair the assessment of safety results or preclude compliance with the study
- not have received another gene therapy

The list above is not exhaustive of all eligibility criteria. To learn more about the **BEAM-301 Clinical Study,** including the exclusion and inclusion criteria, contact clinicalinfo@beamtx.com.

TAKING PART IN THE BEAM-301 CLINICAL STUDY *(cont'd)*

What should I expect?

During study visits, you should expect to undergo certain procedures and tests both before and after you receive BEAM-301. Some of these tests and procedures include the following:


A physical exam


Blood and urine collection for laboratory testing


An electrocardiogram, or ECG, to measure the electrical activity of your heart


Controlled-fasting challenges performed in a hospital


Continuous glucose monitoring throughout the duration of the study


Liver and kidney imaging


Questionnaires to assess any symptoms of GSD1a and your quality of life

People who take part in the BEAM-301 study will be asked to consent to a separate LTFU study at the end of the 2 years.

Your health and safety will be closely monitored throughout the study.

What are the potential risks and benefits of taking part in this study?

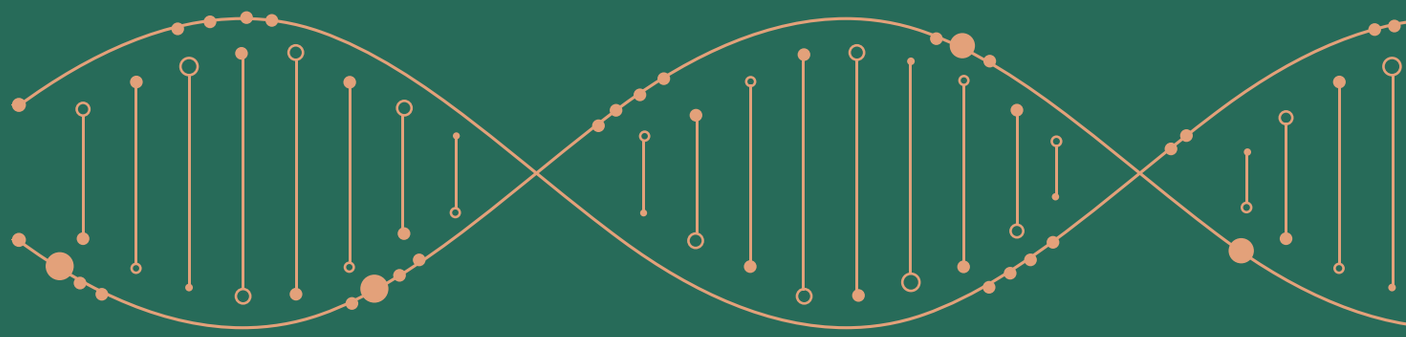
- Every clinical trial may have risks associated with it. It is important to understand these risks before consenting to participate in a clinical trial
- BEAM-301 may help treat GSD1a. It is also possible that your health may worsen or stay the same
- Your participation in this study will help doctors learn more about BEAM-301 and GSD1a. This knowledge may benefit people in the future who have GSD1a

Are the costs related to the study covered?

Your approved travel expenses, and those of your caregiver, to and from the study site may be covered. You will not be charged for the study drug or for any of the tests that you undergo in association with this study. Beam Therapeutics will not pay for routine care or doctor visits that are not part of this study.

THANK YOU FOR CONSIDERING JOINING US!

TOGETHER, WE CAN LEARN MORE ABOUT THE POTENTIAL FOR NEW AND IMPROVED THERAPIES FOR PEOPLE LIVING WITH GSD1a.



Have any questions?

Send an email to clinicalinfo@beamtx.com

This clinical study is sponsored by Beam Therapeutics Inc.