Frequently Asked Questions about the CARAT Study

A clinical research study for patients with Fabry disease and Fabry-related heart involvement.

CARAT

CARdiac **A**ssessmen**T** in Fabry Patients on Venglustat

MAT-CA-2300154

a sanofi study

Thank You for Your Interest in the CARAT Study

The information in this brochure is intended to accompany discussions with the study team. There is a lot to consider, so please make sure to ask the study team for additional information or explanation at any point. Please also talk to your doctor about whether this study may be right for you.

What Is a Clinical Research Study?

Before a medicine can become widely available, it must be tested in a series of clinical research studies to evaluate safety and effectiveness. People who volunteer for studies and receive an investigational medication are closely monitored. Health authorities use the information from studies to decide whether or not investigational medicines should be approved and made available for doctors and healthcare professionals to prescribe to patients as appropriate. There are generally three phases of clinical research studies before health authorities consider granting approval.

Phase 1: Healthy volunteers receive the investigational study drug to help researchers evaluate initial safety and tolerability. Researchers are also looking to see whether the investigational study drug causes any unwanted side effects and understand how the drug is processed by the body. Some Phase 1 studies will include people with the disease after initial results are evaluated from healthy volunteers.

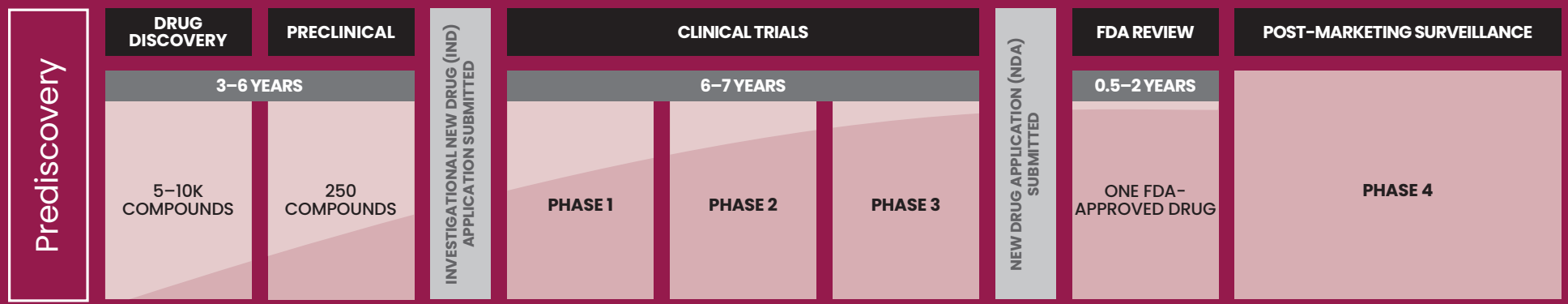
Phase 2: The investigational study drug is given to a small number of people with the disease to help researchers learn more about safety, efficacy, short-term side effects, and optimal dosing. These studies may also evaluate different doses to determine which should be studied further in future studies.

Phase 3: These studies include a larger number of people. They are intended to generate enough information to establish whether:

- The treatment can be safely administered
- The treatment is capable of providing a beneficial effect

The CARAT study is Phase 3.

Traditional Drug Discovery and Development Pathway



What Is the Purpose of the CARAT Study?

Fabry disease is a potentially life-threatening, inherited, multi-systemic disorder caused by reduced or absent alpha-galactosidase A (α-GAL A) activity due to a variant in the GLA gene. Deficient α-GAL A activity results in the accumulation of glycosphingolipid (particularly globotriaosylceramide, also known as GL-3), leading to cellular and organ damage that affects the renal, cardiovascular, and cerebrovascular systems, as well as a reduction in life expectancy and quality of life. We are researching the investigational oral study drug, venglustat, to evaluate its effect compared to standard-of-care treatment (agalsidase alfa, agalsidase beta, or migalastat) on the change in weight of the left ventricle of the heart in patients with cardiac Fabry disease. The standard-of-care treatment options that participants may receive will be those approved by regulatory authorities in their country.

The investigational study drug is taken orally once daily.

Both CARAT study participants and their doctors will know when they start taking the investigational study drug.

Who Can Participate in the CARAT Study?

Approximately 90 patients are expected to participate in the CARAT study globally, and both males and females are eligible. You may be eligible to participate in the CARAT study if you:

- Are between 18 and 65 years old
- Have a confirmed diagnosis of Fabry disease
- Have Fabry-related heart involvement

You will **NOT** be eligible if you:

- Have severe depression or a history of an untreated, unstable major affective disorder within one year of the screening visit
- Have advanced scarring of the heart
- Have advanced kidney disease

Additional criteria will be assessed by a study doctor to confirm your eligibility.

What Happens If I Participate in the CARAT Study?

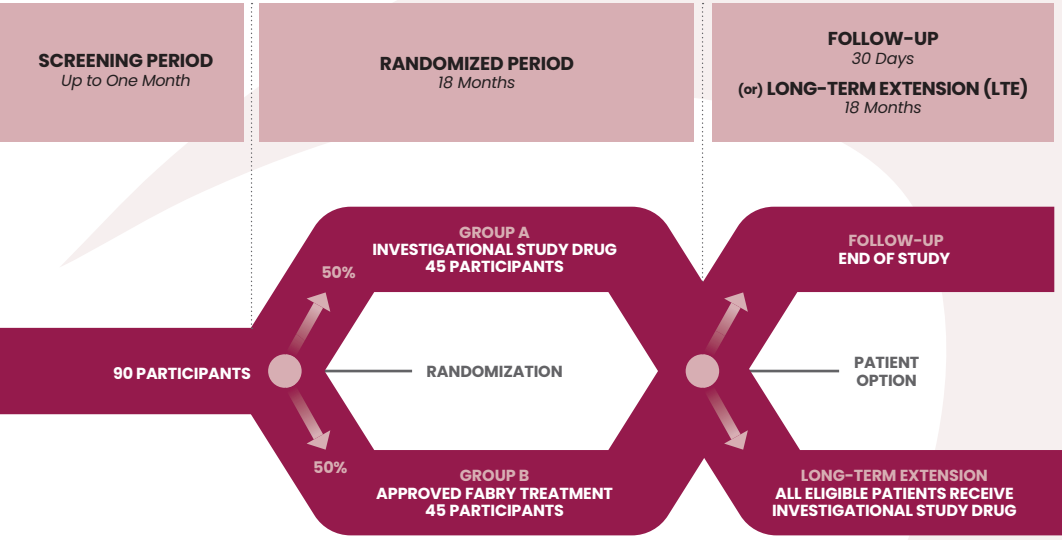
In order to find out whether the investigational study drug is effective versus standard-of-care therapy on cardiac measures and functions, study participants will be randomly assigned to receive either the investigational study drug or standard-of-care therapy. Neither you nor your study doctor will be able to choose which group you are assigned to. At the end of the study, the information gathered from all participants will be compared and analyzed.

The CARAT study is open label. This means that you and the study doctor will both know if you have been assigned to receive the investigational drug or not.

How Long Does Participation in the CARAT Study Last?

Participation in the CARAT study may last up to 36 months:

- **Screening period:** You will have up to one month to complete the screening process to confirm eligibility.
- **Main study/randomized period:** If you are eligible and agree to participate, you will be randomly assigned to receive the investigational study drug or standard-of-care therapy for 18 months. During this study period, you will complete five study visits.



After completion of the main study period, you may have the option, if eligible, to participate in a long-term extension study period.

- **Follow-up period or CARAT long-term extension (LTE) enrollment:** After completion of the main study period, you may have the option to participate in an LTE period.
  - If you decide to participate in the LTE period, you will not complete the follow-up visit of the study period. Instead, you will complete the required assessments to continue into the LTE period, during which you will then receive daily doses of the investigational study drug and help us collect data about its long-term safety and effectiveness. Participation in the long-term extension period is currently estimated to last for up to 18 months.
  - If you decide not to continue into the LTE period, you will have one study follow-up visit 30 days after your last dose. Your total time in the CARAT study if you do not take part in the extension study may last for up to 19 months.

Participation in any clinical research study is completely voluntary. Study participants can also end their involvement at any time, for any reason. Before making a decision to leave, it is important to speak with the study doctor or nurse and complete all required follow-up evaluations.

What Is the Investigational Study Drug?

Venglustat, the investigational oral study drug being researched in the CARAT study, is being studied as a potential oral therapy that is not dependent on the type of Fabry variant. Venglustat is not approved by health authorities such as the US Food and Drug Administration (FDA) and is still under investigation for the treatment of Fabry disease, so its effectiveness and safety have not been established.

What Are the Risks of Taking Part in the CARAT Study?

All clinical trials have possible risks, some of which are known before the study starts. Before you decide to participate, these risks will be explained to you. If you choose to join a study, your health will be closely monitored. Your care will be adjusted if the medical team thinks it's in your best interest. While side effects may occur with any medical product being studied, the safety and well-being of study participants are top priorities for the sponsors, doctors, nurses, and research professionals who conduct clinical trials.

The study team will discuss the potential risks of participating in this study with you in detail so that you can make an informed decision.

Why Do Participants Volunteer for Clinical Studies?

There are no guaranteed benefits to taking part in any clinical research study. Some people participate in clinical research when there are no approved treatments for their disease or when they are not candidates for, or no longer respond to, available treatments. They may also wish to help others by participating in research on the disease.

You should review the informed consent form closely with the study team and make sure that all of your questions are answered before making a decision. Joining a clinical research study is a very personal decision, and only you can decide if you want to participate.

Please write any questions you have about the CARAT study and share them with your doctor or the study team:

To learn more about the CARAT study
and find out if you may be eligible, visit
http://clinicaltrials.gov/ct2/show/study/NCT05280548

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