Introducing the BALANCE Study

a Fabry clinical study balance

For patients with Fabry disease

Thank you for your interest in the BALANCE Study

The BALANCE Study is a clinical research study that will compare the safety and efficacy of an investigational medication (a potential new medication) with an approved medication (agalsidase beta, also known as Fabrazyme) in patients with Fabry disease. The investigational medication is a type of medication called "enzyme replacement therapy," or ERT. The investigational medication has been designed to help the body break down the particular fat (lipid molecule) that builds up in the body's cells because of Fabry disease.

The investigational medication in the BALANCE Study is a new ERT. It is designed to last longer in the body, which might help protect the body's organs from the effects of Fabry disease and reduce symptoms.

What is a clinical research study?

A clinical research study is an investigation that helps us learn whether a potential new medication is effective and safe. All new medications must be tested in a series of clinical research studies before they can be approved and prescribed for use.

- Phase 1 studies look at the investigational medication for the first time in a small group of people.
- Phase 2 studies give the investigational medication to a larger group of patients to find out more about its safety and efficacy.
- Phase 3 studies, like the BALANCE Study, give the investigational medication to a large number of patients to evaluate its efficacy and learn more about its side effects or how it is best used.

Without clinical research studies, no new medicines would be developed and few medical advances would be made. These studies are overseen by local and national regulatory bodies.

What is the BALANCE Study?

The BALANCE Study is a Phase 3 clinical research study involving nearly 80 patients with Fabry disease. It will include Fabry patients who also have worsening kidney function, and who have been receiving agalsidase beta infusions for approximately 1 year.

There will be two patient groups in this study. Who gets which medication will be decided randomly (like flipping a coin). Neither patients nor their study doctors will know what medication they are receiving, but the study team can find out in case of an emergency or any other immediate need.

- Two out of every three patients will receive the investigational medication.
- One out of every three patients will receive agalsidase beta.

All patients will receive the investigational medication or agalsidase beta as an infusion (via a drip into a vein in the arm) every 2 weeks for about 2 years, with one last infusion at the final treatment visit. At the end of the study, patients may have the option of continuing with the investigational medication or receiving it for the first time (if they received agalsidase beta during the BALANCE Study).



What will the BALANCE Study involve?

The study has two periods and up to 56 visits.

Screening period

Approximately 1 month, 1 visit

- The study doctor will ask questions and do some health checks to see if the study is a good fit.

Treatment period

Up to 2 years, 53 visits

- Infusions (1 mg/kg) of either investigational medication or agalsidase beta every 2 weeks and at the last treatment visit.
- Each infusion will take up to 3 hours, but this may be shortened over time.
- Questions, health checks, and other assessments will be done to monitor patient health and learn about the investigational medication.

After the treatment period

- Final health checks will be done 1 and 3 months after the last infusion visit.
- Please note, patients may be able to join a long-term study in which they will all receive the investigational medication, even if they received agalsidase beta during this study.

Who can take part in the BALANCE Study?

Patients with Fabry disease may be able to take part if they:

- are 18–60 years of age
- are experiencing symptoms of Fabry disease, such as pain or clusters of dark spots on the skin known as angiokeratomas
- have been receiving agalsidase beta infusions for approximately 1 year.

For patients interested in taking part, the study team will ask some medical history questions and carry out health assessments at the study center to see if the study is a good fit.

What else do you need to consider?

Taking part in the study is entirely voluntary. Patients can leave the study at any time. Anyone who takes part in the study will be carefully looked after by a team of experienced doctors and nurses. All study medications and study-related healthcare procedures will be provided at no cost. In addition, reasonable travel expenses will be reimbursed.

Patients who take part in the BALANCE Study may or may not benefit from their participation. Patients may have side effects from the investigational medication or agalsidase beta, or their condition could get worse. However, the information gathered from this study may be used to help other patients with Fabry disease in the future.

Want to know more?

The BALANCE Study Team will be happy to give you more information or to answer any questions. You can contact them using the details provided below. Patients may find it helpful to speak with their families or personal doctors about taking part in the study before making a decision.

Thank you for your interest in the BALANCE Study.