The GuardOne Trial: A Phase 1/2 Open Label Clinical Trial in Gaucher Disease Type 1 Key Facts

AVROBIO

GuardOne is designed to evaluate the safety and efficacy of AVR-RD-02, an investigational gene therapy, in individuals with GD1.

Who can participate?

The trial is open to individuals who:

- Are between the ages of 18 and 45 years (depending on the region in which you are participating) at the time of screening;
- Have a confirmed diagnosis of **Gaucher disease type 1 (GD1)** based on genotyping, deficient glucocerebrosidase (GCase) enzyme activity and clinical features consistent with GD1; and
- Have been on stable enzyme replacement therapy (ERT) for a minimum of 24 months, OR have never received ERT or substrate reduction therapy (SRT) or have not received ERT or SRT within the last 12 months.

These are not all the eligibility requirements for GuardOne. Call **1-877-330-5214** or visit ClinicalTrials.gov (study identifier: **NCT04145037**) for a full list of eligibility criteria and additional information.

Study Design

68 weeks (1 year and 4 months)

Screening (up to 8 weeks)	Baseline (1-3 days)	Pre-Gene Therapy (6-8 weeks)	Gene Therapy Infusion (1 day)	Follow Up (52 weeks)
Interested participants are informed of the potential risks and benefits of participation, also called the informed consent process. You will undergo activities to confirm your eligibility, such as a physical examination and providing medical history as well as blood tests and medical imaging tests.	If eligibility is confirmed through screening procedures and tests, investigators perform tests to establish a pre-gene therapy baseline.	Participants are prepared for gene therapy through mobilization, apheresis, and conditioning. • Mobilization helps the bone marrow release stem cells into the bloodstream where they can be selected by a process called apheresis. • Conditioning prepares the body to receive the gene therapy after the mobilized and selected stem cells have been gene modified through the manufacturing process.	Participants receive the gene therapy (AVR-RD-02) via single intravenous (IV) infusion.	Periodic safety and efficacy assessments on going over the course of 52 weeks with up to 17 visits.

The GuardOne Trial: A Phase 1/2 Open Label Clinical Trial in Gaucher Disease Type 1 Key Facts

Phase 1/2 Enrollment Goal: 8-16 patients

Primary Objectives

Evaluate:

- The safety and tolerability of AVR-RD-02, including:
  - Adverse events (AEs) and serious adverse events (SAEs), clinical labs, ECG, vital signs, antibodies, replication-competent lentivirus, insertional site analysis
- The effect of AVR-RD-02 on:
  - Clinical markers of GD1 including the spleen, liver, blood counts, and bone mineral density
  - Biomarkers of GD1 including bone marrow and blood

Secondary and Exploratory Objectives

Evaluate the effect of AVR-RD-02 on clinical measures of Gaucher disease including:

- GCase enzyme activity in the blood
- Patient pain and quality of life

About AVR-RD-02

AVR-RD-02 is an investigational gene therapy being developed for patients with GD1. AVR-RD-02 is still investigational, which means it has not been proven safe or effective or approved by the U.S. Food and Drug Administration (FDA) or any other regulatory agency. Patients should discuss all treatment options, including potential clinical trial participation, with their healthcare provider.

For more information about AVR-RD-02 and the GuardOne trial, **visit AVROBIOGaucherTrial.com** or **call 1-877-330-5214**.