The Guard1 Trial: A Phase 1/2 Open Label Clinical Trial in Gaucher Disease Type 1 - Key Facts

AVROBIO

Guard1 is designed to evaluate the safety and efficacy of AVR-RD-02, an investigational gene therapy, in selected patients with GD1.

Who can participate?

The trial is open to individuals who:

- Are between the ages of 18 and 50 years at the time of screening;
- Have a confirmed diagnosis of **Gaucher disease Type 1 (GD1)**; and
- Have been on stable enzyme replacement therapy (ERT) for a minimum of 24 months, OR have not received ERT or SRT within the last 12 months.

These are not all the eligibility requirements for Guard1. Call **1-877-330-5214** or visit **AVROBIOGaucherTrial.com** or ClinicalTrials.gov (study identifier: **NCT04145037)** for a full list of eligibility criteria and additional information.

Study Design

~72 weeks (1 year and 5 months)

Screening (up to 60 days)	Baseline (1-7 days)	Pre-Gene Therapy (Approximately 8-10 weeks)	Gene Therapy Infusion (1 day)	Follow Up (52 weeks)
Informed of study requirements, risks and potential benefits and to determine if the individual qualifies for the study. Confirm if the individual still meets the requirements of the study.	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 collected and selected by a process called apheresis. • Conditioning prepares the body to receive the gene therapy.	Participants receive the gene therapy (AVR-RD-02) via single intravenous (IV) infusion.	Periodic safety and effect of AVR-RD-02 assessments over the course of 52 weeks with up to 17 visits.

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Phase 1/2 Enrollment Goal: 8-16 patients

Objectives of the study include:

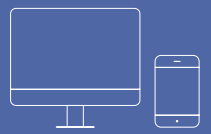
Evaluate:

- The safety of AVR-RD-02 and whether it is well tolerated
- The effect of AVR-RD-02 on:
  - Markers that show the extent or severity of the disease in blood, bone and internal organs such as spleen* and liver
  - Whether AVR-RD-02 can improve the study participant's quality of life

* Individuals who have had their spleen surgically removed can also have the opportunity to participate

About AVR-RD-02

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



For more information about AVR-RD-02 and the Guard1 trial, **visit AVROBIOGaucherTrial.com** or ClinicalTrials.gov (study identifier: **NCT04145037**) or **call 1-877-330-5214**.