



**1 DOSE** • Could it Help Conquer  
• High Cholesterol?

---

**Be Part of the Heart-2 Study**



**heart-2**



# heart-2

## Can you participate?

You may be able to take part if you\*:



Are 18-70 years old (female participants cannot be of childbearing potential)



Have high LDL-C levels despite being on cholesterol lowering medications



Have a diagnosis of HeFH and/or premature CAD



Are not taking a PCSK9 inhibitor medication

\*Additional eligibility criteria apply

# About the Heart-2 Study

The Heart-2 study is a clinical trial for people that have heterozygous familial hypercholesterolemia (HeFH) or premature coronary artery disease (CAD) and high levels of low-density lipoprotein cholesterol (LDL-C). The purpose of the Heart-2 study is to assess the safety and tolerability of the investigational medicine called VERVE-102, and see if it can help lower LDL-C levels with a single dose.



The investigational medicine is designed to permanently turn off production of the PCSK9 protein in the liver. It does this by inactivating the *PCSK9* gene using an approach called gene editing. This may reduce LDL-C levels in the bloodstream. Because gene editing is expected to be a permanent change, it is given just one time through an intravenous (IV) infusion (this means it will be administered into a vein).

---

## What will the study involve?

Approximately 36 people will take part in this study. Participants will be in the Heart-2 study for up to one year after receiving the investigational medicine. After the study is over, you will be asked to join a second, long-term follow-up study to help researchers understand the safety of the medication over a longer period. The study doctor will share the details of this with you.

# The study has three periods:

## **1** Screening Period (up to 3 months)

You will attend screening appointments to determine your eligibility for the trial. During this time, several tests will be performed, including taking blood samples and performing physical examinations.

- You will visit the study site two or more times to determine whether the study is right for you. If you are eligible, you will have the option to join the study.
  - The screening visits need to occur within a three month period.
- 

## **2** Study Treatment Period (about 4 days)

If you qualify and decide to participate, the study treatment period will involve a stay of approximately 4 days/3 nights at the study site, during which:

- You will receive a single dose of the investigational medication as an IV infusion in your arm.
  - During your stay at the study site, the study team will monitor your health and conduct assessments similar to the screening period to check how well you tolerate the investigational medicine. If needed, your stay may be extended for additional monitoring.
- 

## **3** Follow-up Period (about 1 year)

- There will be up to 10 follow-up visits over the year after receiving the investigational medication to continue to monitor your health including the end of study visit.
- After the end-of-study visit, you will continue to participate in a long-term follow-up study to monitor your safety and cholesterol levels. Many of these “visits can be done from home.



## Why should you participate?

Millions of people around the world have been impacted by cardiovascular disease caused by high levels of LDL-C. Many people make lifestyle changes and take cholesterol-lowering medications but still have high LDL-C levels, putting them at increased risk of having a heart attack or stroke.

HeFH is an inherited condition that causes high LDL-C levels. Elevated LDL-C may also lead to premature CAD, which is caused by cholesterol-driven blockage of coronary arteries and a high risk of further complications. CAD is considered premature if a person is diagnosed at age 65 years or younger in women or age 55 years or younger in men. People with high LDL-C associated with HeFH or premature CAD have a high risk of cardiovascular problems such as heart attack or stroke. Lowering LDL-C is the best way to reduce these risks.

---

The investigational medicine may be able to help lower your LDL-C levels for many years or possibly the rest of your life, with a single dose.

# What else do you need to know about the Heart-2 study?

---



The study team will explain the possible benefits and risks of taking part in the Heart-2 study.



If you choose to take part in the study, you can stop participating at any time.



You will be reimbursed for your time participating in this study.



You will be reimbursed for travel costs or other expenses during your participation.



The study medication and study-related tests will be provided at no cost to study participants.



A team of study doctors and nurses will monitor your health carefully during the study.



The study has been reviewed by an Institutional Review Board/Ethics Committee and national health authority, which protects the rights, safety, and well-being of participants.



# heart-2

## How do you get more information?

To find out more, visit the study website

[Heart2study.com](https://Heart2study.com)

Study participation is voluntary. By contacting us, you are under no obligation to take part in the study.

