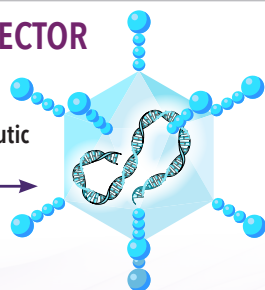


# Understanding AAV Gene Therapy in Cardiovascular Clinical Studies

AAV gene therapy offers the potential for treating cardiovascular disease by addressing the underlying disease mechanisms and is already being used in other conditions such as hemophilia and eye disease.<sup>1,2</sup> AAV gene therapy aims to change the course of a disease by addressing the root cause, which could repair damaged organs, reduce disease-related symptoms, and improve quality of life.<sup>3</sup> These gene therapies are developed to help a wide range of patients, not only those whose illness is caused by inherited genetic conditions. AAV gene therapy utilizes a specially designed tool—using a virus—to deliver healthy copies of genes into the body.

## AAV VECTOR

Therapeutic gene



## What is an AAV Vector?

This gene therapy delivery tool called an AAV vector has been stripped of its original viral genes, so generally it's not known to result in human disease. Scientists use it to deliver healthy copies of a gene into the body's cells or tissue to treat diseases at the source, providing instructions to replace missing or defective genes.

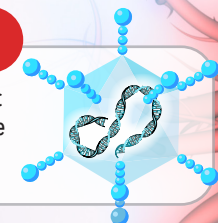
1

A healthy, therapeutic gene is created in the laboratory to treat cardiovascular disease



2

The healthy, therapeutic gene is inserted into the AAV vector



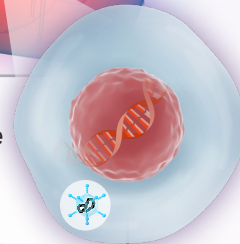
3

The AAV vector containing the healthy, therapeutic gene is delivered directly to the patient's heart tissue



4

The healthy, therapeutic gene works to treat disease at the source by providing instructions to replace missing or defective genes without changing your DNA



## FREQUENTLY ASKED QUESTIONS:

### What Are the Potential Benefits of AAV Gene Therapy?

AAV gene therapy offers the potential for a one-time treatment with lasting effects, aiming to reduce the damage to the heart tissue related to cardiovascular disease, stop or reverse disease progression, and increase quality of life, all while potentially minimizing the side effects associated with ongoing medications.<sup>4</sup>

### Is AAV Gene Therapy Safe?

Like any medical treatment, AAV gene therapies may carry potential risks. Participants in clinical trials undergo detailed medical evaluations and are closely monitored to help identify and manage any potential risks.

### Will My Genetic Makeup Be Altered?

AAV gene therapy creates its own structure to deliver a new working gene that does not become part of your DNA or change your DNA.

### Do I Need Immunosuppression?

- Immunosuppression is not needed for some AAV gene therapy.
- The type of AAV used, the disease, and your body's reaction and immune response determines if immunosuppression is required.

### What is a Clinical Study?

Clinical studies help find better ways to prevent, diagnose, and treat diseases while following strict guidelines to protect participants and ensure reliable results. A group of independent experts, called an Institutional Review Board (IRB) or Ethics Committee, reviews each study to ensure participants are treated fairly and safely. By volunteering, participants help discover new treatments and improve medical care.



**WOMENHEART**

WomenHeart is solely devoted to advancing women's heart health through advocacy, community education, and the nation's only patient support network for women living with heart disease. WomenHeart is both a coalition and a community of thousands of members nationwide, including women heart patients and their families, physicians, and health advocates, all committed to helping women live longer, healthier lives. To learn more visit [womenheart.org](https://www.womenheart.org).



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Additional questions? Please contact [AskFirst@askbio.com](mailto:AskFirst@askbio.com)

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