



ANTIBODY TESTING for Gene Therapy Research

Every trial has requirements for people who can take part in the study. These requirements may include things like age and gender or features of a medical condition.

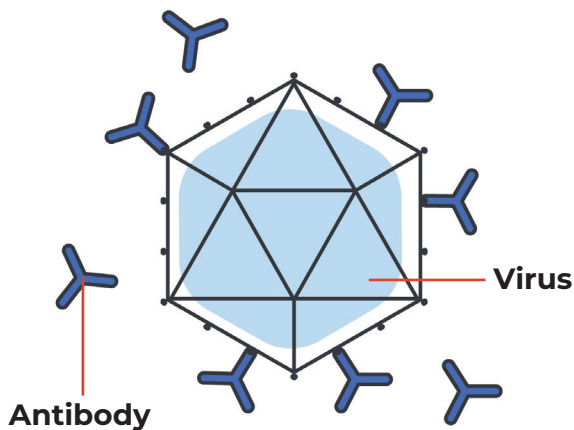
Antibody testing is an important requirement for many gene therapy trials. For people who are interested and eligible for future trials, retesting will occur prior to enrolling into a study.

What is an antibody?

An antibody is a protein made by the immune system, which helps the body recognize foreign bacteria or viruses.²

How does a person make antibodies to a virus?

When the immune system finds a new virus in the body, it makes antibodies to attack and destroy the virus.^{3,4}



The immune system also remembers the virus. If the same virus invades the body again, the immune system makes the same antibodies, called pre-existing antibodies. This can prevent another infection.⁴

What is gene therapy?

Gene therapy is a way of treating diseases caused by genes that are not working the way they should.⁵

For example, in HCM:

- A *MYBPC3* gene that is not working properly can cause HCM.
- Gene therapy may use a vector to deliver a working copy of the *MYBPC3* gene to the heart.

Viruses are often used as vectors because they are efficient at getting into cells. Vectors are typically made using the blueprints of a virus. Only the parts of the virus that help with delivering genetic material are included, while all of the viral genes are removed.⁶


Adeno-associated viruses (AAVs) are one type of virus commonly used as a vector to deliver a working copy of a gene.^{7,8}

- AAVs do not cause any infections or diseases.⁹
- AAVs occur naturally in the environment.⁹
- Many people already have antibodies to AAVs in their body and may not know it.⁹

Why is antibody testing important in gene therapy?

People who have been exposed to an AAV before may not know it, but their immune system will have made pre-existing antibodies to this same virus. If they receive an AAV-based gene therapy, those pre-existing antibodies will attack the treatment before the gene therapy has a chance to work.¹⁰ If this happens, the treatment may not work as intended.

Before joining a gene therapy clinical trial, doctors need to assess the potential for the person's immune system to attack the gene therapy.¹⁰ Doctors use a blood test to measure antibody levels to help decide if a person can participate in the gene therapy trial.^{11,12}



TN-201-0008:
Tenaya Therapeutics is studying levels of AAV antibodies in people with HCM.

To take part in this study, a person must

- Have HCM caused by a change in their *MYBPC3* gene;
- Be 18 to 65 years old; and
- Have symptoms of HCM (e.g., shortness of breath, fatigue, chest pain, palpitations, fainting).

These are not all of the eligibility requirements for the study.

What should I do if I want to take part in the study?

Ask your doctor whether you would be a candidate for this study.

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