Myth or Fact?

Come explore gene therapy for Duchenne

Gene therapy has been researched for decades and is being studied extensively in Duchenne muscular dystrophy. Let's take a look at the science behind it and explore some common facts and myths.

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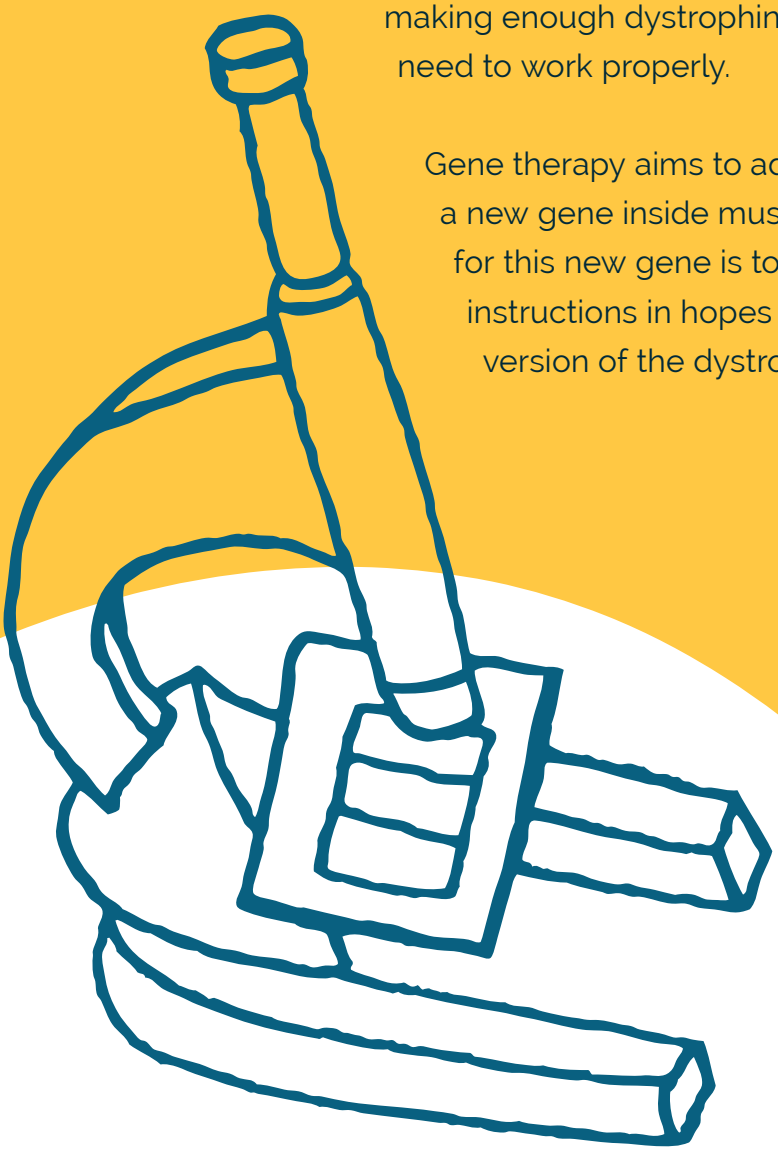
What is gene therapy?

Gene therapy is a way to treat genetic conditions, which are caused by changes (called “mutations”) in the body’s genes.

Duchenne is caused by a mutation in the dystrophin gene. This prevents the body from making enough dystrophin, a protein muscles need to work properly.

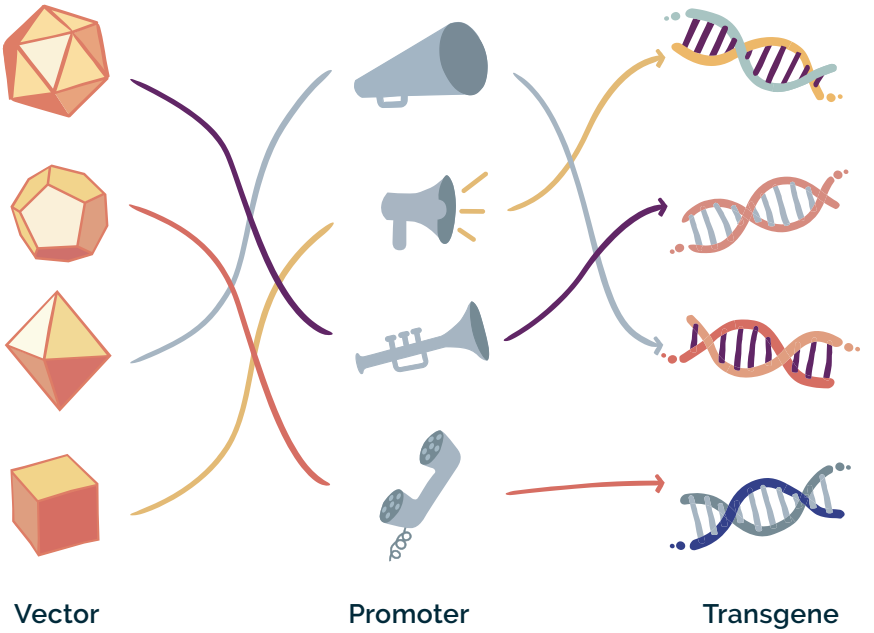
Gene therapy aims to address this by putting a new gene inside muscle cells. The goal for this new gene is to give the cells instructions in hopes of making a working version of the dystrophin protein.



MYTH OR FACT? All gene therapies are different

Fact!

Each gene therapy is unique, even if it's treating the same disease, because each of its main parts—the vector, promoter, and transgene—is different. Think of it like a recipe, where different combinations of ingredients create a different product. Clinical trials help scientists study different combinations and understand how well they might work.



MYTH OR FACT? Gene therapy is a cure

Myth

While some gene therapies may cure diseases, in Duchenne, the goal of gene therapies being studied is to slow or stabilize the disease.

How does gene therapy work?

Each gene therapy is made up of 3 main components: A vector, a promoter, and a transgene

The **vector** aims to deliver the transgene and promoter to the muscle cells

The goal of the **promoter** is to "turn on" the transgene only when it's in the right cells

The **transgene** is the new gene that is intended to provide the instructions needed to make a working version of dystrophin

Many different vectors, promoters, and transgenes are considered when developing a gene therapy. These 3 components are designed to work together to help the body make a new form of dystrophin.

MYTH OR FACT? Vectors are like viruses

Fact!

Vectors are similar to viruses that occur naturally. But these have been modified to prevent them from reproducing or causing an infection once in your body.

MYTH OR FACT? Antibodies don't matter to gene therapy

Myth

Antibodies are part of our immune system that help protect against illness by blocking the effect of viruses and bacteria. The body makes antibodies when exposed to a virus or bacteria so it can fight it off in the future.

Since vectors are similar to viruses, if antibodies related to the vector are present in your blood, they could prevent a gene therapy from doing its job and could cause harmful side effects. But, scientists are researching ways that gene therapy can be given even if a person already has antibodies to the vector.

MYTH OR FACT? Currently, you can have gene therapy only once

Fact!

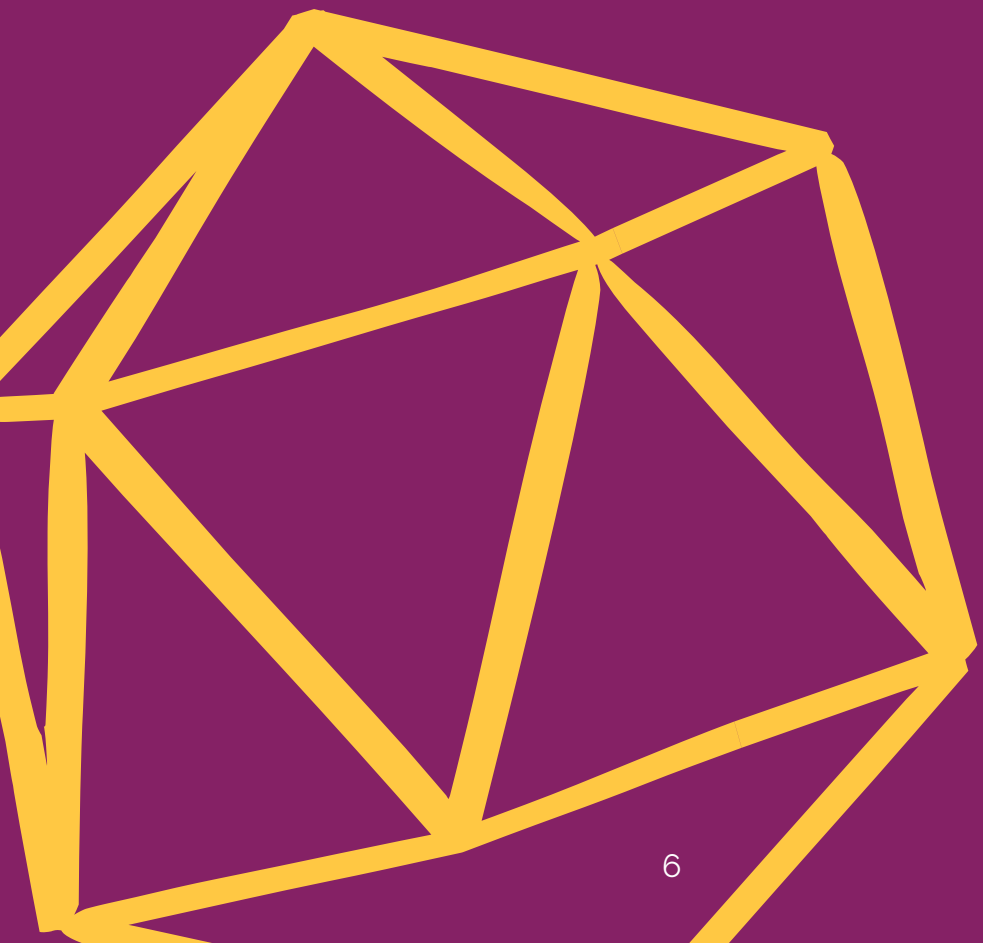
Gene therapy is currently only a one-time treatment. Once you've taken a gene therapy, your body will respond to the vector and your immune system will create antibodies to it. These antibodies may prevent the treatment from working if given a second time.

Who is eligible for gene therapy?

Because every gene therapy program is unique, each has different guidelines about who can receive treatment. This is called eligibility criteria.

Two key criteria that broadly apply are confirming a diagnosis (verified with a genetic test) and testing for antibodies in your blood related to the vector. If you don't have a confirmed diagnosis or have too many vector antibodies, you may not be eligible for treatment.

Many things go into determining eligibility, and the criteria may change as we learn more, so it's always best to talk to a doctor for the most up-to-date information.



MYTH OR FACT?

Gene therapy is intended for many mutations

Fact!

Gene therapy is not designed to be limited to 1 specific genetic mutation.

MYTH OR FACT?

Eligibility for clinical trials and approved products is always the same

Myth

Clinical trials are set up with very specific requirements for who can participate to ensure consistency when the results are collected. These requirements are tightly controlled. If the FDA approves the product, the guidelines for who could receive it would be included in the Prescribing Information. Talking to a doctor is the best way to find out whether a gene therapy is the right option.

Looking for more?

- You can find in-depth information on Duchenne gene therapy at **Duchenne.com/genetherapy**
- If you're looking for more information about gene therapy across diseases, visit the *American Society of Gene and Cell Therapy* at **patienteducation.asgct.org**

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