

Myths and Facts About Gene Therapy

Gene therapies use genetic materials to treat or prevent disease. Genes contain the instructions needed to make the proteins our bodies require.

Alteration of any gene that results in missing or abnormal instructions may impact how our bodies work. Gene therapy involves the use of a vector as a sort of envelope to deliver necessary genetic materials (the “package”) to restore or improve the protein-making instructions.

While gene therapy offers hope for many individuals living with neuromuscular disease,

a lot remains unknown about this type of therapy, including the long-term risks. Patients and caregivers must thoroughly understand these unique risks and challenges before deciding to pursue gene therapy.

The lists below contain facts and unproven myths. Understanding the facts can help you make the most of gene therapies.



DISCLAIMER: This document is meant to inform and educate the community. The information presented is not intended to replace discussions with your healthcare provider and is not and should not be considered to be medical advice. Please consult with your healthcare team and/or insurance company for information specific to you.

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Myths

These statements are false:

- Gene therapy is a cure.
- Gene therapy is simple.
- Gene therapy can be reversed.
- Gene therapy is guaranteed to show beneficial results.
- Gene therapy treatments are not yet of proven clinical value.
- Gene therapy is available to treat everyone with a neuromuscular disease diagnosis.
- Any physician can prescribe gene therapy treatment safely.

Facts

These statements are true:

- Researchers have not determined how long the effects of gene therapy treatments will last nor the extent of benefit over time. Factors that may impact the effectiveness and durability of gene therapies include age, treatment dose, and disease progression.
- Gene therapy targets specific cells. A person's basic genetic composition remains unchanged; only the cells targeted by the therapy change.
- Not everyone will be eligible for gene therapy. Considerations of age, function, pre-existing antibodies, etc., may determine eligibility.
- Early studies show that gene therapy may slow the progression of some diseases.
- Receiving gene therapy may affect access to other types of treatment later.
- Long-term effects of gene therapy remain unknown due to the limited data available.
- Gene therapy affects individuals differently and can cause severe side effects or even death in some.
- Gene therapies require months-long monitoring and treatment of side effects after infusion of the treatment.
- Gene therapy is irreversible and can result in permanent changes to the body.
- Over the past two decades, MDA has invested over \$125M in gene therapies for many neuromuscular diseases — ranging from basic science research to clinical trials.
- MDA's investments have led to the development of clinical-ready gene therapies for diseases such as spinal muscular atrophy (SMA) and Duchenne muscular dystrophy (DMD), with many more in the drug development pipeline.
- MDA-funded researchers conducted the first human gene therapy clinical trial for muscular dystrophy in 1999.

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MDA's involvement in gene therapy

MDA has invested over \$125M in the development of gene therapy (GTx) for neuromuscular diseases over the past 20 years. With new gene therapy drug approvals in the pipeline, MDA is here to help facilitate access and provide support and education to the neuromuscular disease community.



By Phone:

1-833-ASK-MDA1 (1-833-275-6321)

By Email:

ResourceCenter@mdausa.org

Gene therapy support from MDA

MDA Gene Therapy Support staff are available Monday through Friday, 9 a.m. to 5 p.m. CT. Answers to inquiries can be expected within one to two business days. MDA services are available only in the US. If you live outside the US, we may be able to connect you to muscular dystrophy groups in your area.

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