

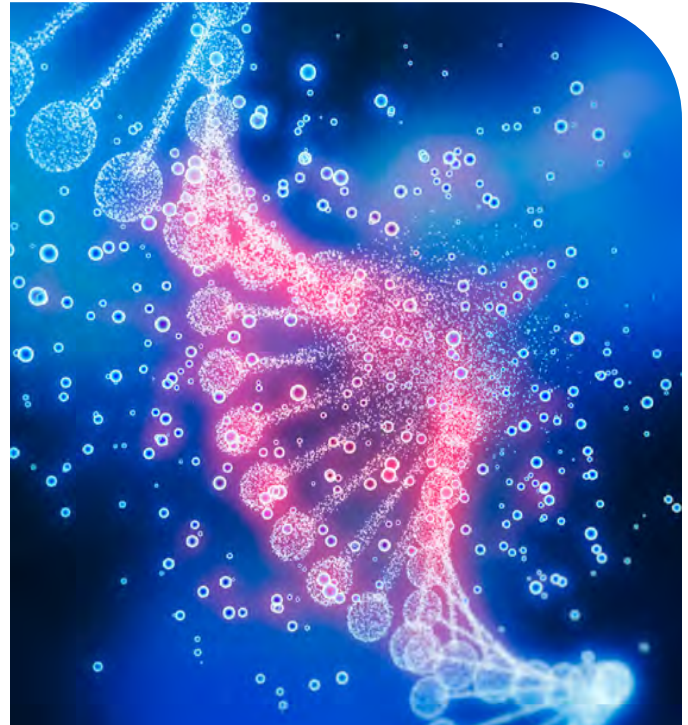
# A Guide to Gene Therapy

For Individuals and Families

## What to Expect Before, During, and After

**Gene therapy** is a new type of treatment that introduces specific genetic material into affected cells to treat or slow down the progression of a genetic disease. The process of receiving gene therapy treatment involves complex care that typically extends over several months, so patients and families need to understand each step well. Please note that while this document breaks down the overall gene therapy process into before, during, and after-administration steps, some may happen at the same time, and many are tailored to each patient and will differ from one patient to the next.

Before receiving gene therapy treatment, it is important to ask any questions you may have about the therapy and how it fits into your comprehensive treatment plan. Potential risks — some of which are very serious — are associated with gene therapy treatment. Your



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care team will explain the therapy and discuss possible risks and benefits in detail. Overall, receiving gene therapy is a multistep process with many intricate details, but your doctor and healthcare team will guide you through each step to ensure a safe experience.

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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.

# A Guide to Gene Therapy

## Before administration

- Confirm genetic diagnosis.
  - If undiagnosed, complete genetic testing.
  - Confirm patient compatibility (age, mutation type) with the drug label.
  - Understand risks.
  - If applicable at your site, read informed consent in its entirety.
- Complete baseline labs needed for insurance submission, screening, and optimizing care after the infusion.
- Complete additional baseline testing, as applicable.
- Ask the physician and care team any outstanding questions.
- Determine post-administration and caregiver plans, including overnight hospital stay plans and plans for the family to remain near the treatment center, etc.
- Complete pre-infusion medications, as applicable.

## Day of administration

- Follow all instructions your care team provides, including the need for additional pre-treatment medications.
- Receive a single infusion, which can last from minutes to a few hours, performed by healthcare providers at the treatment facility.

## After administration

- Continue to monitor for issues related to receiving the infusion.
- Complete post-infusion medications, as applicable, to manage any potential side effects.
- Receive liver and heart function monitoring as specified by the treatment care team.
- Continue all labs needed to assess overall health.
- Sometimes, patients may need a peripherally inserted central catheter (PICC) placed to help manage therapy after the infusion.
- Manage viral vector shedding. Shedding is the process by which the body eliminates the viral vectors used in gene therapy. Ensure all caregivers take appropriate precautions to avoid contact with the viral vector from patient secretions.
- Complete any additional labs up to 36+ weeks post-administration, as prescribed by the physician, including additional monitoring as the need arises during the post-monitoring period.
- Monitor for any changes and report them to the treatment care team.
- Understand the importance of monitoring your condition; be in close contact with the administering care team, ensuring regular follow-up and communication.
- Continue all needed therapy and care for the underlying genetic disorder — gene therapy can be very effective but does not eliminate the need for ongoing care.

## 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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## Important considerations

- It is important to understand that filling out a patient “start form” does not mean you are pre-authorized and/or approved by your insurance company to receive gene therapy treatment; the start form is just the beginning of that process.
  - Receiving pre-authorization from your insurance company can take weeks and sometimes months to gather all the necessary information and complete the paperwork.
  - Insurance denials throughout the process are normal and to be expected. For help navigating insurance denials, please check out **MDA’s Access to Coverage: Insurance** workshop, contact the MDA Resource Center, or work with your care team.
- Not everyone will be eligible to receive gene therapy. Limitations by age, function, pre-existing antibodies, etc., are considered for eligibility and can differ between insurance plans.
  - Eligibility to receive gene therapy may change. It is important to confirm with your administering physician and health insurance provider that you are eligible for treatment.
  - If you are not eligible to receive gene therapy, discuss with your physician other treatment options that may be available for you.
  - Antibody testing may occur multiple times during this process depending on your administering physician, health insurance provider, and the product label (e.g., the label may require a negative test within 30 days of treatment).
  - Being eligible to receive gene therapy does NOT mean you are next on the list at your treatment center. The process is complex and may take time. Treatment centers will have a limited capacity to treat patients safely. It will take time to treat all eligible patients.
  - Do not fax any healthcare information to your administering physician’s office. It is best to email via a trackable, secure email, ideally through the electronic medical records system for that healthcare system.
  - Pharmaceutical companies do not have access to patient health record information from administering clinicians. Insurance companies do have access to medical information.
  - Gene therapy may have adverse reactions and can lead to death. Make sure you understand the risks and how they can be optimally minimized or managed.
  - It is important to determine an end-of-life plan before gene therapy administration to help advance research and treatments for future patients. This can be done by determining participation in autopsy and/or body donation. Complete an autopsy consent form in advance of administration.

### By Phone:

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

### By Email:

ResourceCenter@mdausa.org

# A Guide to Gene Therapy

## 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.



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## REFERENCES

American Society of Gene and Cell Therapy  
[patienteducation.asgct.org/gene-therapy-101/gene-therapy-approaches](http://patienteducation.asgct.org/gene-therapy-101/gene-therapy-approaches)

Food and Drug Administration  
[fda.gov/consumers/consumer-updates/how-gene-therapy-can-cure-or-treat-diseases](http://fda.gov/consumers/consumer-updates/how-gene-therapy-can-cure-or-treat-diseases)

This document was prepared with input from Barry J. Byrne, MD, PhD, University of Florida; John W. Day, MD, PhD, Stanford University; Natalie Goedeker, MSN, CPNP, Washington University in St. Louis; and Julie Parsons, MD, Children's Hospital of Colorado.

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