

The Impact of Gene Therapy on Families

Gene therapy seeks to correct the underlying cause of a genetic disease by introducing genetic material into a person's affected cells. For example, a gene therapy might involve inserting a healthy copy of a gene to replace a faulty or missing gene.

The practice of administering gene therapy in clinics is relatively new, and experts are still defining its benefits and associated risks. Although a gene therapy infusion takes no more than a few hours, the process of receiving a genetic treatment involves complex considerations before and after the infusion that typically extend over several months and can affect multiple aspects of family life.

It is important for patients and families to understand the potential consequences of gene therapy on their specific situation. Below are a few of the areas for consideration.

Siblings

Because many neuromuscular diseases are genetic, they often affect multiple family members. Some families may find themselves having to consider gene therapy treatment for multiple children.

In some cases, one sibling might be eligible to receive gene therapy while other siblings are not eligible. In other situations, multiple siblings are eligible to receive gene therapy, but the logistics might not work to receive treatment at the same time. These situations can lead to difficult decisions. Note that the risk-benefit considerations for children within the same family can be different based on factors such as age, disease progression, and preexisting antibody levels.



If you have more than one child who is ELIGIBLE to receive gene therapy, consider the following:

- Consult with your physician and care team to determine the timing for each child to receive treatment.
- Develop a post-administration monitoring plan and schedule with your care team.
- Ask your care team about vector shedding and how to manage it.
- Manage expectations on post-treatment outcomes — potential benefits and side effects may differ among siblings.
- If you are traveling for an infusion, plan for extended time away from home to be near the treatment center.
- Develop plans for family members who are not traveling as well as those who are.

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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If you have a child who is eligible to receive gene therapy and one or more children with the same diagnosis who are INELIGIBLE, consult with your care team, a social worker, and/or a mental health professional to:

- Ensure your communication with each child is age appropriate.
- Determine what future treatments are expected for each child.

Managing vector shedding

Gene therapy uses a vector to deliver new genetic material into cells of the human body.

Currently, viral vectors, such as adeno-associated virus (AAV), are used in neuromuscular disease gene therapies. Nonviral vectors, including lipid nanoparticles and biopolymers, are being developed for future use in neuromuscular diseases.

When a person receives gene therapy using a viral vector, they will excrete virus byproducts in their urine, feces, sputum, blood, and secretions from sores or wounds for a period following the treatment. Family members should avoid contact with the patient's body fluids during this time to prevent developing antibodies to the viral vector. This is especially important if other family members are hoping

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to receive gene therapy, as having antibodies to AAV may make them ineligible for it.

Consult with your care team to make a plan to manage vector shedding. Precautions might include having the person who received gene therapy use a separate bedroom and bathroom while they are shedding to reduce others' exposure to the viral vector.

Considerations for caregiving and monitoring

Receiving gene therapy is a complex and highly involved process. Families must be prepared to commit to being near the treatment center and care team for weeks after the infusion to ensure they are closely monitored for negative reactions. Those traveling for the infusion should be prepared to be away from home for an extended period.

Your care team will discuss monitoring and negative reactions to look for during the first days, weeks, and months in addition to monitoring for potential long-term effects of the gene therapy. The individual will likely need lifelong monitoring and management of functional changes related to the underlying genetic disorder.

Documented side effects from patients who have received gene therapy include nausea, vomiting, loss of appetite, fever, malaise, kidney and liver injury, blood clots, and inflammation of skeletal muscle and the heart. There is a range of responses to gene therapy, and clinicians are unable to predict who will experience negative side effects.



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Extended stays at gene therapy centers/sites

Overnight stays will be determined by the healthcare team. During clinical trials of gene therapies, overnight stays are common to allow researchers to capture detailed measures of acute effects that can help determine optimal dosing and other aspects of treatment. However, once the therapy is commercially available, healthcare institutions may be strongly incentivized to administer gene therapies in outpatient facilities with close monitoring during and immediately after the infusion.

In general, patients receiving gene therapies will remain near the treatment center for weeks after the infusion so they can be closely monitored for negative reactions. Potential side effects of gene therapy can occur within days to months after the infusion, and patients should immediately tell their care team about any changes to get appropriate care, including hospitalization if necessary.

Familiarize yourself with the area around the treatment facility to help make your family comfortable while away from home for an extended period. Suggestions include locating grocery stores or favorite restaurants for meals. In addition to other essentials, be sure to pack anything that will help make being away from home more comfortable, such as a favorite blanket, pillow, slippers, and electronic devices for music, movies, TV shows, and gaming.

Financial assistance and travel

Some pharmaceutical companies offer financial or other support for travel to and from designated treatment centers for gene therapy infusions. Travel support may include car rentals, flights, hotel stays, meals, and caregiver coverage. Eligibility criteria and the degree of support, are determined by the pharmaceutical company.



MDA cannot provide financial assistance for gene therapy administration or travel assistance to treatment centers. For more information about support options, consult your care team and the pharmaceutical company that manufactures the gene therapy.

Your emotional support network

You will benefit from setting up an emotional support network you can connect with during the gene therapy process. It is also important to communicate with extended family and friends and help manage their expectations. The person receiving gene therapy typically will need to minimize contact with extended family members and friends — especially anyone who might have an infectious disease — for weeks before and after the infusion.

The care team at your treatment center, including the social worker and psychological counselors, can help your whole family prepare for this important period of treatment.



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Share and read through the following documents together:

- **Understanding Gene Therapy – FAQs for Individuals and Families**
- **Myths and Facts About Gene Therapy**
- **A Guide to Gene Therapy — What to Expect Before, During, and After**

Other items to consider:

- If you are traveling for the treatment, determine who is attending and who is staying home to care for other family members, including pets.
- Make sure to communicate with your child's school administration and teachers regarding extended absences and accommodations that might be needed. Collaborate with the school to find creative solutions, such as participating virtually, receiving assignments in advance, alternative assignments, or tutoring.



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. We continue to support gene therapy programs for a range of neuromuscular diseases from preclinical to clinical stages of development. 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.

Gene therapy support from 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.

Call: **1-833-ASK-MDA1 (1-833-275-6321)**

Email: **ResourceCenter@mdausa.org**

Visit: **mda.org/care/gene-therapy-community-support**

This document was prepared with input from John W. Day, MD, PhD, Stanford University, and Julie Parsons, MD, Children's Hospital of Colorado.

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