



Gene Therapy Planning Checklist

Gene therapy is a 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 of the process.

This Planning Checklist should be completed by caregivers with the help of their child's medical providers (Care Team). Each section should be reviewed before starting gene therapy.

Please note that while this document breaks down the overall gene therapy process into before, during, and after treatment steps, some may happen at the same time, and many are tailored to each patient and will differ from one patient to the next.

PART I

Preparing for Treatment

- Schedule initial appointment with Care Team.

Date of appointment: _____

What to bring/prepare: _____

- Fill prescriptions and begin any medications as prescribed by your Care Team to prepare for gene therapy treatment.

Medications: _____

- Discuss with your Care Team if any immunizations need to be updated before their immune system is suppressed in preparation for treatment.

Immunizations needed: _____

Understand any out-of-pocket costs such as co-pays, deductibles, medications, travel, lodging, etc.*

Notes: _____

Complete baseline labs as required. This may include antibody testing.

Date of appointment: _____

Labs needed: _____

Complete pre-infusion instructions as required.

Notes: _____

Understand the risks and potential complications. If applicable at your treatment site, read the informed consent in its entirety.

Ask physician and Care Team any outstanding questions.

Notes: _____

Consider building a support network for you and your family. Talk to your Care Team's social worker or ask your Care Team for referrals to social and emotional support resources.

Notes: _____

Work with your Care Team to understand the risks of gene therapy treatment and how those risks can be optimally minimized or managed. Gene therapy may have adverse reactions including the possibility of death. In this situation, a consent for autopsy will be discussed.

Notes: _____

**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 rental, flights, hotel stays, meals, caregiver coverage, etc. Eligibility and criteria, as well as the degree of support, are determined by the pharmaceutical company.*

PART II

Preparing for Day of Treatment

- Understand and follow all instructions for day-of treatment (including the need for any additional pre-treatment medications).

When to arrive: _____

Where to park: _____

Who to call (if needed): _____

Additional instructions: _____

- Map out how to get to the treatment center; plan your departure time accordingly taking into consideration weather and traffic.

Estimated travel time: _____

We need to leave by: _____

PART III

Preparing for Post-Treatment Care

- Pack clothes, medications, snacks, toiletries, comfort items (stuffed animal, blanket), phone chargers, tablets, photo identification, insurance cards, etc.

- Determine possible length of stay both at the hospital and, if needed, at a nearby hotel or other lodging for monitoring. Arrange for lodging.

Estimated number of nights at hospital: _____

Estimated number of nights at nearby lodging: _____

Lodging options: _____

- If traveling to a treatment center, familiarize yourself with the surrounding area. Consider locating grocery stores, gas stations, restaurants, etc.

Notes: _____

- Determine who will stay at home with siblings and pets (if applicable).

- Discuss any extended absences or accommodations needed with the child's school or daycare, if needed. Prepare for school absences in advance by requesting any schoolwork from the child's teacher or determining alternative ways to participate.

Notes: _____

- Understand what post-infusion medications might be needed to manage side effects and identify a pharmacy to fill these prescriptions (if applicable).

Medications needed post-infusion: _____

Pharmacy contact: _____

- Understand what post-infusion labs might be needed and when. Determine how the labs will be completed.

Labs needed and when: _____

Location/Contact for Lab: _____

- Determine a plan for managing viral vector shedding. This might include having gloves and masks available for all caregivers and frequent sanitation of shared living areas. It might also include isolating siblings who have not yet received gene therapy treatment but may in the future.

Precautions we will take: _____

- Ensure all caregivers understand the importance of post-treatment monitoring and educate them on what side effects to watch for and how to seek help in an emergency.

Symptoms to monitor: _____

Report side effects and other concerns to:

Contact information: _____

When to contact 911: _____

- Continue all needed therapy and care for the underlying genetic disorder. Gene therapy can be very effective, but it does not eliminate the need for ongoing care.

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.

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.

Our Care Team

Physician:

Phone/Email:

Nurse:

Phone/Email:

Cardiologist:

Phone/Email:

Pulmonologist:

Phone/Email:

Social Worker:

Phone/Email:

Hospital Admissions:

Phone/Email:

Infusion Center:

Phone/Email:

Pharmacy:

Phone/Email:

Other contacts:
