Navigating Insurance: Gene Therapy

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 is complex and involves many important considerations along the way.

This guide provides the neuromuscular disease community with insight into the insurance approval process, proactive steps to take throughout the process, and options for appealing a denial. The information and tips in this guide cannot guarantee insurance approval of a treatment. It is important to work closely with your healthcare provider throughout the process as they will have insight into your/your loved one's specific situation.

MDA's Gene Therapy Support Team is available to answer questions and identify resources throughout the process. Contact MDA's Gene Therapy Support Team at 1-833-ASK-MDA1 (1-833-275-6321) or at resourcecenter@mdausa.org.

To learn some of the basics of insurance coverage, take the MDA Access to Coverage: Insurance Workshop at MDA.org/care/access-workshops.

MDA® Muscular Dystrophy Association

Part I: Obtaining Insurance Approval

STEP 1: Understand Your Insurance

First thing you need to do before pursuing gene therapy treatments is to understand your health insurance coverage.

Each insurance plan includes a Summary of Benefits Coverage (SBC) document that itemizes the care and services that are covered as well as what is not covered. You should request a copy of your complete Policy Documents from your employer or directly from your insurance company.

Important items/descriptions to look for in your SBC and/or Policy documents:

Deductibles, Co-Pays, Coinsurance, and out-of-pocket maximums: It is important to understand how costs are shared between you and the insurance company.

Benefit exclusions: Statements about what your plan will not cover. Look for “gene therapy” and/or “experimental treatments”.

Criteria for coverage/medical policy: An outline or description of specific criteria needed for the insurance company to cover certain treatments or procedures. Examples are: age, diagnosis, antibody testing results within certain limits, genetic testing, history of medications, etc.

Medical Necessity: Review your Policy Documents for your insurance provider’s definition of “medical necessity” or “medically necessary services”. This definition is important to reference when seeking coverage for treatment.

Medical and Prescription Coverage: Review you coverage for both the treatment (medication) and the procedure (i.e., infusion, hospital stays, etc.). The medication, if covered, will most likely be under your medical coverage rather than your prescription coverage but it is good to understand both.

Tip

Highlight the specific phrases within the plan document that might pertain to your situation. This will make the key statements easier to find later.

STEP 2: Request an Advocate or Case Manager

Call your insurance company and request an advocate or case manager be assigned to you. An advocate/case manager can help you understand and navigate your insurance policy. They should help you utilize your insurance to the fullest extent and can be an additional point of contact throughout the process.

Tip

Take notes on any phone calls you have with the insurance company. Write down the date of the call and the name of the person you spoke with as well as a brief summary of what was discussed.

STEP 3: Work with your Healthcare Provider

Your healthcare provider/prescribing physician most likely has a team member who focuses on obtaining approvals from insurance for treatments and procedures. Ask who this person is and how to contact them. Work with this person to:

- **Obtain all necessary paperwork**. This may include:
  - Prescription from your provider
  - Letter of medical necessity. See example on page 5 of this document.
  - Test results, which may include some or all of the following:
    - Genetic test results
    - Antibody testing
    - Blood work
    - Urine tests
    - Respiratory tests
    - Cardiac function tests
    - X-rays
    - Motor Milestone Assessments
    - Functional Rating Scales
    - Clinic notes

- **Understand the logistics** of receiving treatment including testing, timelines, and scheduling.
- If necessary, **navigate the appeal process**.

Tip

Your insurance company can also provide you with an Authorized Representative Form for you to sign allowing your healthcare provider to manage the appeal process.

STEP 4:Connect with the Pharmaceutical Company

The pharmaceutical company that is providing the medication/product most likely has a patient support program or case manager available to support you through the process of obtaining access.

The pharmaceutical company can provide:

- Information on how to obtain treatment.
- Comprehensive support service programs. This may include assistance with travel, lodging, or other needs associated with treatment.
- Resources to help navigate insurance coverage.
- Financial assistance programs if your insurance doesn't cover treatment.
- Events or opportunities to connect with others.

While your healthcare provider is your primary point of contact during the process, it is still important to connect with the pharmaceutical company providing the medication in case they can offer additional resources or information.

STEP 5: Submit Documentation

Submission of documentation will be completed by your healthcare provider, but it is important that you are aware of, and included in the process. Your healthcare provider will submit the required documentation which may include a letter outlining exactly how you or your loved one meets the criteria for coverage. This is known as a letter of medical necessity.

It is highly recommended that the letter of medical necessity be clearly detailed.

- **Explain how patient meets criteria**. A table sampled below can be an effective way to demonstrate meeting criteria:

SAMPLE LETTER OF MEDICAL NECESSITY

Attn: Insurance Company A
Re: Treatment X Prior Authorization
J-Code: JXXX
Service Date: 1/5/24 to 3/5/24

Patient Information
Name: John Smith
Date of Birth: 12/12/2019
Insurance: Company A
Member ID: 12345-678

Dear Insurance Company A:

I am writing to request approval of Treatment X for John Smith. This therapy is medically necessary for John to treat his diagnosis of Duchenne muscular dystrophy.

John meets your company's clinical policy for this drug. Attached documentation supports this. (Link to policy if available)

Insurance Company A Criteria	**Patient Information**
Member is male and between 4-5 years of age.	*John is a 4 year-old male.*
Member has a genetically and clinically confirmed diagnosis of Duchenne muscular dystrophy.	*John has a confirmed mutation of exons 48-50. Genetic report is attached.*
Member is ambulatory.	*John remains ambulatory as demonstrated by his physical therapy exam (attached).*
Member has an anti-AAVrh74 total binding antibody level <1:400 confirmed by ELISA	*John's has an anti-AAVrh74 titer <1:400 (lab result attached)*
Member does not have any of the following: a) deletion in the exon 8 and/or 9 of the DMD gene; b) prior use of gene therapy	*John's deletion is not in exon 8 or 9 and there is no history of gene therapy in this patient.*

If you have any additional questions regarding this patient or Treatment X, please do not hesitate to contact me.

Sincerely,

Your healthcare provider

STEP 5: Submit Documentation (continued)

- If the patient does not meet criteria, the next step is to make a compelling case for coverage. At this stage, do not reiterate prescribing information.
  - Write short, factual statements.
  - Use data to back-up your statements.
  - Avoid using emotional wording.
  - Tailor it to the individual
  - If you are attaching additional information such as test results, data reports, notes from your provider, etc., make sure to list those documents in the letter.
  - Approach it as a team effort, getting input from physicians, nurse practitioners, physical therapists, genetic counselors, etc. as needed.

Tip

Ask your healthcare provider for a copy of all documentation sent to the insurance company on your/your loved one's behalf. Ask them to include the date the documentation was sent and the method of delivery (i.e., email, fax, mail).

Part II: Determination from the Insurance Company

Receiving an Approval

Take a moment to celebrate! Then get started on the following next steps:

- Save a copy of the approval letter/email in your records.
- Contact your healthcare provider for next steps.
- Understand any out-of-pocket costs (i.e., co-pays and deductibles).
  - If co-pays or deductibles are cost prohibitive, look at organizations such as The Assistance Fund that can help cover the cost of some treatments depending on diagnosis.
  - Contact MDA's Gene Therapy Support Team for support in researching other resources.
  - Try to be patient. Keep in mind that it may still take some time to set up the actual dosing of the therapy. The hospital administering the therapy will likely need to get a single case agreement with the insurance company. This is an agreement of payment terms for how much and when the insurer will pay the hospital for the therapy. Ideally, insurers and hospitals have contracts in place already, but for newer therapies it may take several weeks to get the contract in place. It may also take a few weeks for the therapy to be ordered and arrive at the hospital.

Receiving a Denial

Receiving a denial can be very upsetting, especially when there are very few treatments for neuromuscular disease. There are still avenues you can take to try to reverse the denial.

It is important to understand why the treatment was denied. Sometimes the reason for denial will be explained in a document titled **Explanation of Benefits (EOB)** from your insurance company. Other times you may need to call your healthcare provider and insurance company to learn more.

Denials typically fall into one of the following categories:

Reason for Denial	>	What to do About it
Does not meet plan criteria Sometimes the insurance company may base their criteria on the requirements from the clinical trial and not on the drug label. In clinical trials, criteria may be stricter to ensure the drug reaches the intended target and endpoints. Clinical trials often require that participants be very similar – such as specific genetic mutations or steroid requirements. Receiving FDA approval comes with the determination of the label that outlines who and how the drug can be used. The label may or may not be as strict as the clinical trial criteria.	>	Work with your healthcare provider to educate the insurance company on the label requirements. Your provider may need to provide additional data or proof that the treatment meets your insurance company's definition of "medical necessity", including the consequences of not receiving the treatment. If your insurance company states that the treatment is not the "lowest cost option", work with your provider to outline why this specific treatment is the best option for you/your loved one's care.
Misinterpretation of policy, clinical data, and/or both At times, the insurance company may misinterpret clinical data or may misunderstand how the policy is written, such as mixing up the "and"/ "or" in their policy. The insurance representative reviewing the request for coverage may have misunderstood the difference between deletion and duplication or may have misread the AAV antibody titers.	>	In this case, it may be best to request a peer-to-peer review directly. See page 8 for more information about requesting a peer-to-peer review.
Therapy is "experimental" or "investigational" While the treatment has been approved by the FDA, some insurance companies may consider treatments that went through an **accelerated approval process** as "experimental" and may state that it is "not medically necessary for any indication."	>	If this is the reason for denial, it may be best to go straight to an external review. To learn more about the external review process, see page 8.
Benefit Exclusion This is an administrative denial. Employer self-funded plans can exclude coverage for a broad range of items. Your plan might have a generalized "gene therapy" exclusion. This requires override by the employer, which precludes the use of excess insurance or stop-loss insurance, and can be very expensive for the employer.	>	Contact your employer and ask for plan documents to see if gene therapy is a benefit exclusion. Speak with your employer's human resources department to see if they are able and willing to override the exclusion.

What is a self-funded plan?

Health insurance through an employer can be self-funded or fully insured.

A **self-funded** plan means that the employer pays the healthcare services covered by the plan. Many large companies, government agencies, and school districts have self-funded plans. Claims are reviewed and managed by a third-party administrator (TPA). The TPA may share a brand name with an insurance company, but the employer still pays the claims, not the insurance company.

In a **fully insured plan**, the insurance company charges your employer a premium to take on the financial responsibility of paying the claim.

Part III: Navigating the Appeal Process

It is important to understand that you may have to appeal the insurance company's decision multiple times. Be persistent! The process of appealing a denial can take time.

Your policy documents should outline how to initiate an appeal. The denial letter you receive may also outline the process. An **internal review** means that the appeal is reviewed internally by the insurance company. An **external review** is when a third-party, outside of the insurance company, reviews the appeal.

1. **First Level Appeal: Peer-to-Peer (Internal)**
   Your healthcare provider will speak with a medical reviewer at the insurance company to explain the medical need for the treatment. The goal is to prove that your request meets the insurance guidelines, and that the denial was incorrect.

2. **Second Level Appeal: Medical Director (Internal)**
   Your appeal is reviewed by a medical director at your insurance company who was not involved in the original decision to approve or deny your coverage. This is to prove that your request is within the coverage guidelines and should be accepted.

3. **Third Level Appeal: Independent External Review (External)**
   An independent external reviewer and a healthcare provider who specializes in neuromuscular care will review your appeal and determine if coverage will be approved or denied. The third-party independent reviewer is often managed by your state's insurance regulatory agency. You can find your state's insurance regulatory agency at naic.org.

Generally, insurance companies have 30 days to respond to an appeal for treatment that has not yet been received. If your healthcare provider feels that the denial of your/your loved one's claim could be life-threatening, request an expedited appeal.

Tip

Establish response deadlines. An insurance company typically has 30 days to respond to an internal appeal request. If you receive a denial from the internal appeal, you typically have 60 days to request an external appeal. When you speak with insurance representatives, healthcare providers, etc. make sure to request deadlines and dates for when you can expect a response.

Hard (Terminal) Denials

If all levels of appeal have been exhausted and the treatment is determined to fall outside of the scope of the insurance policy, it is considered a hard or terminal denial. This is certainly a disappointing situation. Even still, there are actions you can take:

- **Secondary Medicaid:** Depending on your state, you/your loved one may qualify for Medicaid as a secondary insurance. In some cases, Medicaid might cover the treatment. To learn more about Medicaid in your state, visit Medicaid.gov
- **Other insurance options:** If your spouse's employer offers a different insurance plan, consider switching to their plan. This can be complicated depending on enrollment periods and qualifying events but could be an option to explore. The Health Insurance Marketplace might also be an option as these health plans cannot have benefit exclusions. Open enrollment may still be a barrier though depending on your situation. Find your local Health Insurance Marketplace at localhelp.healthcare.gov/.
- **Rally your network**: Talk with your family, friends, co-workers, and neighbors to drum up support.
    - Start a petition (i.e., change.org) and share it on social media. Ask others to share it.
    - Contact local news stations and newspapers. Ask them to share your story. MDA offers a Media Toolkit that can be used to appeal to different media channels. MDA can customize it to your situation. To request a copy, reach out to the Resource Center at 1-833-ASK-MDA1 or resourcecenter@mdausa.org.
    - Contact your local state representative and other politicians.

Summary

Navigating health insurance can be time-consuming and stressful, especially for treatments that are complex and new. It requires a lot of patience and organization. Educate yourself on your/your loved one's health insurance plan. Partner with your healthcare team to navigate the process together. You will run into challenges but be persistent. You are your, or your loved one's, best advocate.

MDA is also here to help you every step of the way. Connect with MDA's Gene Therapy Support Network by calling 1-833-ASK-MDA1 (275-6321) or resourcecenter@mdausa.org. Gene Therapy Support Specialists can help you with finding information, resources, and support.

Acknowledgement

This guide was developed with input from Michael Storey, PharmD, MS, Medication Use Strategist at Nationwide Children's Hospital.

References

NAIC
Healthcare.gov

Resources

Below are just some of the resources available to support individuals and families pursuing gene therapy. For additional resources, please contact MDA's Gene Therapy Support Network at 1-833-ASK-MDA1 (275-6321), email resourcecenter@mdausa.org or visit MDA.org/care/gene-therapy-community-support.

MDA Resources

- MDA Access Workshops
  - Access to Coverage: Insurance
  - Access to Gene Therapy
  - Access to Coverage: Approved Treatments
- Print-Ready Educational Materials
  - Myths and Facts about Gene Therapy
    - English I Espanol
  - A Guide to Gene Therapy
    - English I Espanol
  - Understanding Gene Therapy
    - English I Espanol
  - The Impact of Gene Therapy on the Family
    - English
  - Gene Therapy Planning Checklist
    - English

Financial Resources

- The Assistance Fund
- Help Hope Live
- Patient Advocate Foundation

Health Insurance Information

- Health Insurance Marketplace
- Little Hercules Foundation
- National Association of Insurance Commissioners
  - Consumer Information and Resources
  - State Insurance Regulatory Agencies
- Patient Advocate Foundation
  - Understanding Your Health Insurance Policy Documents

Glossary

Accelerated Approval
This program, instituted by the Federal Drug Administration (FDA), allows for earlier approval of drugs that treat serious conditions, and that fill an unmet medical need based on a surrogate endpoint.

Appeal
A request for the insurance company to reconsider their decision to deny coverage.

Benefit exclusion
A treatment, procedure, or test not covered by the insurance policy.

Coinsurance
This is your percentage of the cost of a health care service. Your plan approves what the cost of the service will be and shares the cost. You usually start paying this after you meet your plan's deductible.

Co-payment
A set cost determined by your plan that you pay to visit a provider or to receive specific medical services. The amount can vary depending on each provider or service you receive. A co-pay typically does not count toward your deductible, but it may count toward your out-of-pocket maximum.

Deductible
A pre-determined amount that you pay for certain health care services before your insurance plan begins to cover expenses. Once you meet your deductible, your plan will start to pick up a portion, or all of the medical expenses for the remainder of the year. Deductibles vary depending on the health plan.

Drug Label
Labeling for prescription medicines is required for all FDA-approved prescription medicines. Such labeling is:

- Proposed by the drug company,
- Reviewed by the FDA, and
- If acceptable, approved by the FDA. If the labeling for a new medicine is found to be unacceptable by the FDA, the medicine will not be approved by the FDA.

All prescription medicines have Prescribing Information and carton and/or container labeling and many, but not all, have labeling for patients or caregivers.

Explanation of Benefits (EOB)
This document is sent by your insurance company to outline how expenses for a specific doctor's visit, procedure, or other medical care is shared between you and the insurance company. It is not a bill. If medical care was denied, the EOB may state the reason for the denial.

External Review
A medical reviewer who is independent and outside of the insurance company and a healthcare provider who specializes in neuromuscular care reviews the appeal and determines if coverage is approved or denied.

Gene Therapy
Gene therapy introduces new genetic material into a patient's body to treat or slow the progression of a genetic disease.

Internal Review
A medical reviewer or medical director within the insurance company reviews the appeal.

Glossary

Letter of Medical Necessity
The written explanation from the treating physician describing the medical need for services, tests, treatment, or other medical care for the insured.

Medical Necessity
A term used in health insurance documents to describe the coverage offered by the plan. The definition can vary between insurance policies. Your policy's definition can usually be found in the policy documents and/or in state law.

Out-of-pocket Maximum
The most you will have to pay for covered health care services during your plan's year. Once you meet the maximum determined by your plan, your health plan will pay for 100% of your covered medical costs for the remainder of the year.

Peer-to-Peer Review
Your healthcare provider will speak with a medical reviewer at the insurance company to explain the medical need for the treatment. The goal is to prove that your request meets the insurance guidelines, and that the denial was incorrect.

Policy Document
A formal document that details all of the information regarding the insurance contract, including terms and conditions. It may also be referred to as a plan document or a plan booklet.

Self-funded plan
A type of plan usually present in larger companies where the employer itself collects premiums from enrollees and takes on the responsibility of paying employees' and dependents' medical claims. These employers can contract for insurance services such as enrollment, claims processing, and provider networks with a third-party administrator, or they can be self-administered.

Summary of Benefits Coverage (SBC)
An itemized list of the care and services that are covered by an insurance plan, as well as a list of what is not covered.

Join the Community

Instagram: @mdaorg

Facebook: MDAorg

LinkedIn: Muscular Dystrophy Association

Twitter: @MDAorg

Advocacy Twitter: @MDA_Advocacy

YouTube: YouTube.com/MDA

TikTok: @mdaorg

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