

Evidence in Focus:

Delandistrogene Moxeparvovec Gene Therapy in Individuals with Duchenne Muscular Dystrophy

Summary for patients and care partners

The American Academy of Neurology (AAN) assembled a panel of expert doctors and researchers to review the current studies of delandistrogene moxeparvovec (Elevidys) for treating Duchenne muscular dystrophy (DMD). This summary reviews the conclusions of their research, which was published in May 2025.

What is DMD?

DMD is a genetic condition that causes muscle weakness. It is an X-linked condition that typically affects males with differences in the *DMD* gene. Symptoms of DMD usually appear when a child is between 2 and 4 years old and get more severe over time. Dystrophin is an important protein that helps maintain muscle health and is missing in people with DMD.

What is delandistrogene moxeparvovec (Elevidys) and how does it work?

Delandistrogene moxeparvovec (which goes by the trade name Elevidys and is made by the pharmaceutical company Sarepta) is a one-time gene therapy approved for the treatment of males with DMD. This type of gene therapy involves injecting the person with a smaller version of the gene called microdystrophin to partially replace the missing dystrophin. It is packaged into a viral vector to deliver it to the muscle cells, where it produces microdystrophin.

How is the treatment administered?

Delandistrogene moxeparvovec is given intravenously (into a vein) at specialized treatment centers. Receiving the therapy will be an outpatient procedure for most people. After the therapy is administered, the person will need to have multiple follow-up doctor visits and blood tests in the first few months to monitor for side effects related to the treatment. People receiving delandistrogene moxeparvovec are required to also take a steroid medication (usually a higher dose of the steroid they are already taking). For those on vamorolone, their doctor will also prescribe prednisone starting the day prior to the infusion.

What does the research say?

Current research shows that delandistrogene moxeparvovec may possibly slow the decline of motor function (the ability to control movements) by a small amount in people with DMD. However, it's difficult to know how much of this benefit is due to the gene therapy and how much is due to the person's steroid medication. The research so far has mostly measured the effect on motor function, so we don't know how the therapy might affect other symptoms of DMD (such as heart and lung function). It also isn't clear if the small benefit observed will impact day-to-day activities, quality of life, or lifespan.

Only people of male sex were included in the studies. The studies show different effects in different age groups. Delandistrogene moxeparvovec has not yet been studied in older patients, so we don't know whether it benefits people over the age of 7. It has also not been studied in females with differences in the *DMD* gene and clinical symptoms.

Some people in the studies experienced muscle inflammation and increased weakness, heart inflammation, low platelet count (which could affect blood clotting), or liver injury. As of the time this article was published, one person has died from liver failure after receiving the treatment.

Who is eligible for treatment?

To be eligible for treatment with delandistrogene moxeparvovec, a person must have a diagnosis of DMD (excluding people with deletions of exons 8 and 9), be negative for antibodies specific to the treatment, and be at least 4 years old.

Does the treatment have any other positive effects?

Based on the current research, we don't know if delandistrogene moxeparvovec will extend a person's life or improve their quality of life. The therapy does not cure DMD.

Is it possible to receive multiple doses of delandistrogene moxeparvovec?

No, at this time, people can only receive one dose in their lifetime.

Will delandistrogene moxeparvovec affect other drugs a person is taking?

So far, researchers don't know how the therapy may interact with other medications a person is taking. Other than the requirement to be on a steroid medication, there are currently no restrictions on what other medications a person can be on while receiving treatment with delandistrogene moxeparvovec. Researchers have indicated that people may be at an increased risk for serious immune responses during and shortly after treatment. So, the person receiving treatment should be up to date on all recommended vaccines before the infusion.¹

Does receiving this therapy make a person ineligible to receive other DMD treatments or participate in future clinical trials?

If a person has already been treated with a different gene therapy, they are not eligible for treatment with delandistrogene moxeparvovec. Receiving delandistrogene moxeparvovec may also make people ineligible for future clinical trials of treatments for DMD, particularly those that use viral vectors for therapy delivery.

What should I ask my doctor?

- Am I eligible to receive delandistrogene moxeparvovec?
- What are my expected risks and benefits of delandistrogene moxeparvovec?
- Will my insurance pay for the treatment?
- Where is the nearest treatment center where I could receive delandistrogene moxeparvovec?

The full Evidence in Focus can be found at [AAN.com/guidelines](https://www.aan.com/guidelines).

To learn more about brain health, go to [BrainandLife.org](https://www.brainandlife.org).

¹ Mendell, Jerry R. et al. Practical Considerations for Delandistrogene Moxeparvovec Gene Therapy in Patients With Duchenne Muscular Dystrophy. *Pediatric Neurology*, Volume 153, 11 – 18.
[https://www.pedneur.com/article/S0887-8994\(24\)00014-6/fulltext](https://www.pedneur.com/article/S0887-8994(24)00014-6/fulltext)

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