



This family education sheet gives you information about Elevidys® gene therapy for Duchenne Muscular Dystrophy (DMD).

### Key points:

- On June 22, 2023, the U.S. Food and Drug Administration (FDA) approved a new treatment for DMD called Elevidys (delandistrogene moxeparvovec-rokl).
- Elevidys is a gene therapy that delivers a small, working copy of the dystrophin gene to muscle cells, providing the ability to produce the micro-dystrophin protein in their muscles. This can help to improve muscle function and delay symptoms of DMD.
- Elevidys is a 1-time treatment given through an intravenous (IV) infusion (an IV needle placed in the vein) in Boston Children's Hospital's Outpatient Infusion and Day Treatment clinic. Children don't usually need to stay overnight after the therapy.
- Elevidys is approved for boys and men ages 4 and older who have had a genetic test confirming that they have DMD.

## What is Elevidys gene therapy?

- Elevidys (delandistrogene moxeparvovec-rokl) is a new gene therapy used to treat DMD. DMD is a genetic condition that weakens the muscles throughout the body because the dystrophin protein is missing.
- It's a prescription treatment made available by the pharmaceutical company Sarepta.
- The FDA approved this treatment on June 22, 2023.

- Something must carry the gene into the cell. This is called a **vector**. Viruses are often used as vectors because they're very good at getting inside of cells.
- We use the AAVrh74 viral vector to transport the micro-dystrophin gene to replace the missing or broken dystrophin gene.
- Once replaced, the new micro-dystrophin gene makes micro-dystrophin protein, which improves your child's muscle function and quality of life.

## Who can be treated with Elevidys?

- Boys who have DMD who have had a genetic test confirming that they have DMD may be eligible for this treatment.
- Boys who are going to get Elevidys take a higher dose of steroids for a few months after the treatment.

## Will the AAVrh74 vector make my child sick?

No. Scientists deactivate (turn off) the part of the virus that causes infection. They keep the part of the virus that's good at getting into the cell. The inactive virus will stay in your child's urine (pee), stool (poop) and saliva for a few days after getting the treatment. We call this **viral shedding**.

## How is Elevidys given?

- Elevidys is a 1-time infusion treatment given through an IV. An IV is a needle placed in a vein.
- Elevidys is given in Boston Children's Outpatient Infusion and Day Treatment Clinic (which we call the Infusion clinic). It's given by a registered nurse who's trained in giving this type of treatment to children.
- Elevidys is given over 60 minutes. After the infusion, your child will stay in the infusion clinic to be watched for at least 3 hours.

## What are the risks?

The risks of taking Elevidys are:

- Inflammation (swelling) of the liver or increased risk of injury to the liver. Your child's medical team will closely watch for this and advise what dose of corticosteroid medicine they should take to lower this risk.
- Inflammation of the heart muscle (myocarditis)
- Inflammation of the muscle tissue (myositis)
- Thrombocytopenia, which is a low number of platelets in the blood
- Not being able to easily fight off infections due to taking a higher dose of corticosteroid medicine (which is required for 3 months after Elevidys)

## How does Elevidys work?

- The genes in our DNA are the "building blocks" of the cells that make up all of our bodies. Gene therapy fixes genes that are missing or not working the right way.
- Elevidys delivers a small, functional copy of the dystrophin gene – the gene responsible for causing DMD. Here's how:

## What should I watch for before and after infusion with Elevidys?

- **Yellow coloring of the skin or the whites of the eyes** – this may be a symptom of liver inflammation.
- **Excessive fatigue, dizziness, chest pain** – this may be a symptom of myocarditis.
- **Muscle pain with weakness** – this may be a sign of myositis.
- **Viral respiratory infections:** It's important to watch your child for viral respiratory infections, like a cold or the flu, before and after Elevidys treatment. Corticosteroids can lower the ability to fight infections.
  - Keep your child away from people who have symptoms of an illness, like a cold, coughing, a fever or flu-like symptoms. If someone in your home gets sick, try to keep that person in a different room from your child.
- **Increased bruising or bleeding:** After Elevidys treatment, your child's blood platelet count may go down. Call your child's doctor or nurse right away if you notice that your child has more bruising or bleeding than normal.

## What happens after the treatment?

Before you leave the hospital, talk with your child's medical team about the plan for follow-up care.

- After the treatment, your child must stay close enough to the hospital to be able to return for weekly blood work and close monitoring.
- Your child will have labs (blood work) weekly for at least 2 months after treatment.
- Your child will take a higher dose corticosteroid for at least 90 days after getting Elevidys. This helps to lower the risk of liver inflammation.
- **Contact your doctor right away if your child misses a dose of the corticosteroid.**
- Your child's medical team will tell you when it's safe to lower your child's corticosteroid dose.
- Your child will be seen at Boston Children's Duchenne Muscular Dystrophy Program for appointments to check on the therapy's progress.

## How should I handle my child's pee and poop?

For a short amount of time, small amounts of AAVrh74 may be found in your child's bodily waste, pee and poop. It's important to follow these safety steps:

- Use good hand hygiene when coming into direct contact with bodily waste for up to 1 month after getting Elevidys. If you can't wash with soap and water, use an alcohol-based hand rub.
- Seal disposable diapers in trash bags and throw them out with your regular trash.

## What do I need to know about vaccinations and Elevidys?

Since your child must take a corticosteroid after treatment, it may affect the vaccines they can get. Talk with your child's medical team to find out which vaccines are safe for them during this time.

## How do I know if my health insurance covers Elevidys?

Because this therapy is still new, it's not yet clear how many insurance providers cover this therapy.

We encourage you to talk with your health insurance company and the medical team.

Boston Children's Financial Services staff will guide you through the insurance approval process.

## Who will be part of my child's Elevidys care team?

Your child's care team for this treatment is made up of:

- Infusion nurses
- Neurologists
- Pharmacists
- Financial Services staff

## Contact us

Duchenne Muscular Dystrophy Program

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*This family education sheet is available in Spanish.*