

Elevidys

delandistrogene
moxeparvovec-XXXX

injection for intravenous use

**TREATMENT
GUIDE**

DRAFT

Introduction to ELEVIDYS

ELEVIDYS is a prescription gene therapy used to treat individuals with Duchenne muscular dystrophy (DMD) who have a confirmed mutation in the dystrophin gene and are ambulatory. It is not a cure.

ELEVIDYS is a single-dose intravenous infusion – readministration is not recommended. There are several essential steps required both before and after infusion day, including ongoing monitoring.

This guide provides information on:

page	
4	Important Safety Information
6	Who is eligible for ELEVIDYS
7	Preparing for treatment
8	Infusion day
9	After treatment
11	ELEVIDYS treatment reminders

This guide is not intended to replace the advice of your child's doctor. Please follow the advice and guidance of your child's doctor.

What is ELEVIDYS?

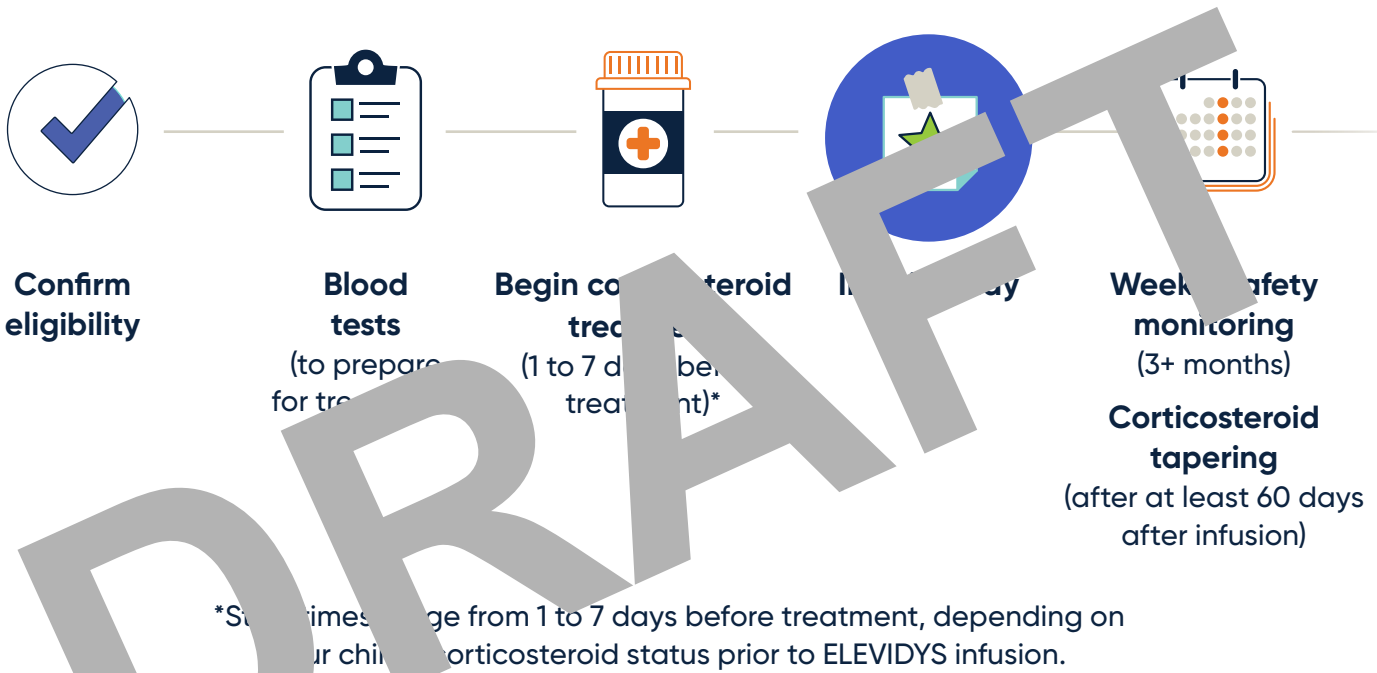
ELEVIDYS is a prescription gene therapy used to treat ambulatory individuals with Duchenne muscular dystrophy (DMD) who have a confirmed mutation in the dystrophin gene. ELEVIDYS was approved under accelerated approval. Accelerated approval allows for drugs to be approved based on a marker that is considered reasonably likely to predict a clinical benefit. ELEVIDYS treatment increased the marker, ELEVIDYS dystrophin, in skeletal muscle in patients. Verification of a clinical benefit may be needed for ELEVIDYS to continue to be approved. Individuals with certain type of mutations, any deletion that includes exons 9-13 in the *DMD* gene should not receive ELEVIDYS.

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Treatment with ELEVIDYS

There are 5 main steps to treatment with ELEVIDYS



If you have questions throughout your child's treatment journey, talk to your doctor.

Always follow your doctor's instructions for your child's treatment-related corticosteroid use, blood tests, and follow-up office visits.



SareptAssist Case Managers are available.

1-888-SAREPTA (1-888-727-3782)

Monday through Friday, 8:30am – 6:30pm ET

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Important Safety Information



What is ELEVIDYS (delandistrogene moxeparvovec-xxxx)?

ELEVIDYS is a prescription gene therapy used to treat ambulatory individuals with Duchenne muscular dystrophy (DMD) who have a confirmed mutation in the dystrophin gene. ELEVIDYS was approved under accelerated approval. Accelerated approval allows for drugs to be approved based on a marker that is considered reasonably likely to predict a clinical benefit. ELEVIDYS treatment increased the marker, dystrophin, in skeletal muscle in patients. Verification of a clinical benefit may be needed for ELEVIDYS to continue to be approved.



Who should not receive ELEVIDYS?

Individuals with certain type of mutations, any deletion that includes exons 1-11 in the *DMD* gene should not receive ELEVIDYS.



What is the most important information to know about ELEVIDYS?

ELEVIDYS can cause a decrease in enzyme levels and acute serious liver injury.

Patients will receive oral corticosteroid before and after infusion with ELEVIDYS and will be on corticosteroids for 14 days. Patients should monitor liver function. Contact the patient's doctor immediately if the patient's skin and/or whites of the eyes appear yellowish, or if the patient has a decrease in appetite or vomits it up.

Administration of ELEVIDYS may be delayed in patients who have acute liver disease until the condition is resolved or under control. Patients with pre-existing liver impairment or chronic liver infection may be at higher risk of acute serious liver injury.

A life-threatening reaction involving a type of muscle damage called immune-mediated myositis happened in a clinical study patient one month after receiving the ELEVIDYS infusion. Caregivers should contact the patient's doctor immediately if the patient experiences symptoms including severe muscle weakness, difficulty swallowing, breathing, or speaking due to weakened vocal cords.

The patient's doctor will conduct blood tests to determine platelet counts, as well as a blood test to evaluate a cardiac protein called troponin-I which can detect damage to muscle cells in the heart. Increased troponin-I has been seen following ELEVIDYS infusion, including a serious case.

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Important Safety Information (continued)



What is the most important information to know about ELEVIDYS? (continued)

Patients need to have blood tests to ensure that they do not have antibodies that may prevent them from being able to receive ELEVIDYS as introducing the gene therapy could increase the risk of a severe allergic reaction. Treatment with ELEVIDYS is not recommended for patients who have high antibodies to the vector, part of gene therapy used to deliver ELEVIDYS.

Due to the need to follow a corticosteroid regimen before and after ELEVIDYS, caregivers should be aware that signs of an infection before or after infusion such as coughing, wheezing, sneezing, runny nose, sore throat, or fever could lead to more serious complications. Caregivers should contact the patient's doctor immediately if they see any symptoms suggestive of infection.



Are there any considerations for vaccinations schedules and ELEVIDYS?

Patient's vaccinations should be up to date according to current immunization guidelines. Vaccinations should be received at least 2 weeks prior to starting the corticosteroid regimen that is required before receiving ELEVIDYS.



Are there any precautions that need to be considered when handling patient's bodily waste?

Vector shedding of ELEVIDYS occurs primarily through body waste. Patients and caregivers should use good hand hygiene when coming into direct contact with patient body waste. Precautions should be followed for one month after receiving ELEVIDYS.



What are the possible or likely side effects of ELEVIDYS?

The most common side effects that occurred in patients treated with ELEVIDYS were vomiting and nausea.

Other reported side effects included increased liver function tests, fever, and decreased platelet counts.

The safety information provided here is not comprehensive. Talk to the patient's doctor about any side effects that bother the patient or that don't go away.

You are encouraged to report negative side effects of prescription drugs to the FDA. Visit www.fda.gov/medwatch or call 1-800-FDA-1088. You may also report side effects to Sarepta Therapeutics at 1-888-SAREPTA (1-888-727-3782).

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Who is eligible for ELEVIDYS

Before treatment with ELEVIDYS, your doctor will need to take the following steps to check that your child is eligible:



Ambulatory

Your child's doctor will confirm that your child is ambulatory.



Genetic test

Your doctor will review your child's genetic test to confirm a mutation in the *DMD* gene in order to be eligible for treatment.

ELEVIDYS cannot be used in people with a deletion that fully includes exons 9 to 13 in the *DMD* gene.



Antibody test

Your doctor will perform blood tests to measure the presence of antibodies to the ELEVIDYS vector (AAVrh74). Your doctor will use a specific test provided by Sarepta.

Individuals may naturally develop antibodies to the vector (AAVrh74) in ELEVIDYS. This means that the immune system would recognize the vector, potentially causing an immune response after the ELEVIDYS infusion. If your child's immune system has anti-AAVrh74 antibodies above the required threshold, your child may not be eligible for ELEVIDYS.



Enrollment Form

You and your child's doctor must complete and submit an ELEVIDYS Enrollment Form.

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Preparing for treatment

The following will need to be completed in advance of your child's infusion day:



Vaccination

If possible, your child's immunizations should be up-to-date with current immunization guidelines prior to starting the corticosteroid regimen required before ELEVIDYS infusion. Vaccinations should be completed at least 4 weeks prior to the start of the corticosteroid regimen.



Weight check

Your doctor will confirm your child's weight to help determine the right dose of ELEVIDYS.



Preinfusion tests

Your doctor will perform blood tests with a clinical team before the infusion day. These tests will evaluate kidney function, platelet count, and troponin-I levels. Your doctor will repeat these laboratory tests after your child's infusion as part of safety monitoring. If your child has a liver disease, your doctor may postpone ELEVIDYS administration until it is resolved or controlled.



Corticosteroid regimen

To help reduce the risk of an immune response to ELEVIDYS, your doctor will prescribe an ELEVIDYS treatment-related corticosteroid regimen prior to receiving ELEVIDYS. Your child will need to continue to take this course of corticosteroids for at least 2 months (60 days) after infusion, as recommended by your doctor. This regimen is in addition to any other oral corticosteroid treatment that your child may currently be taking.

YOUR CHILD:

WHEN TO START ELEVIDYS TREATMENT-RELATED CORTICOSTEROID REGIMEN:

✓ Currently takes corticosteroids

1 day before infusion day

✗ Does not currently take corticosteroids

1 week before infusion day

Always follow your doctor's instructions about your child's ELEVIDYS treatment-related corticosteroid regimen, and talk to your doctor if you have any questions or concerns.

Please see additional [Important Safety Information](#) on page 4 and accompanying full [Prescribing Information](#).

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Infusion day

ELEVIDYS is given as a single-dose intravenous infusion



Administration

A small tube inserted by a needle will deliver the therapy into a vein in your child's arm.



Duration

The actual infusion will last approximately 1 to 2 hours, but your treatment visit may be as long as a full day, based on your doctor's clinical judgment.

IMPORTANT NOTE

Tell your doctor if you have any signs or symptoms of infection before ELEVIDYS administration. If your child has signs of infection, the infusion may be postponed, due to the increased risk of a serious immune response.

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After treatment



Postinfusion corticosteroid regimen

Immune responses to the AAVrh74 vector can occur after administration of ELEVIDYS. To reduce the risk of an immune response, continue with the ELEVIDYS-related corticosteroid regimen prescribed by your doctor.

Your child should take this course of corticosteroids for at least 60 days. Your doctor will tell you when to adjust, taper and/or stop this treatment-related corticosteroid regimen.

Note: If your child's blood test results or clinical exams show liver function abnormalities following ELEVIDYS infusion, your doctor may increase your child's corticosteroid dose temporarily.

Contact your doctor if your child needs a corticosteroid dose or vomits it up.

Due to corticosteroid use, infections (cold, flu, gastrointestinal issues, ear infection, bronchitis, etc) before or after infusion could lead to more serious complications. Call your doctor if you see any symptoms that are suggestive of infection (eg, coughing, sneezing, runny nose, sore throat, or fever).



Postinfusion monitoring

Your child will need weekly monitoring tests for 3 months. Your doctor will monitor the following:

• **Platelet function** through blood tests and clinical exams (weekly for 3 months)

- **Blood test results** (for 1 month); your doctor will monitor your child's platelet counts (weekly for the first 2 weeks) and troponin-I levels to monitor for cardiac muscle damage (weekly for 1 month)

Follow your doctor's instructions for ongoing monitoring.

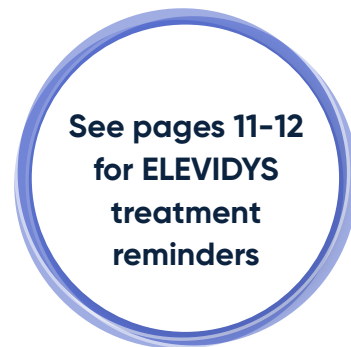
Your doctor may continue monitoring for longer periods of time.



Hand hygiene

Small amounts of ELEVIDYS may be found in your child's body waste (urine, feces, saliva), so proper hand hygiene is important for 1 month after ELEVIDYS infusion.

Hand washing is one of the most effective ways to prevent the spread of germs. Clean hands can stop germs from spreading from one person to another. Follow these steps each time you come into contact with body fluids and waste.



See pages 11-12
for ELEVIDYS
treatment
reminders

Please see additional **Important Safety Information** on page 4 and accompanying full **Prescribing Information**.

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After treatment (continued)

Safety considerations for ELEVIDYS

Side effects can happen, so it's important to know what to look out for.



Contact your doctor immediately if:

- Your child's skin and/or whites of the eyes appear yellowish, as this may be a sign of increased liver enzymes
- Your child experiences any unexplained increased muscle pain, tenderness, or weakness, including severe muscle weakness, difficulty swallowing, breathing, or speaking due to weakened vocal cords, as this may be a sign of a severe immune reaction in the muscles known as immune-mediated myositis



In clinical trials, the most common side effects of ELEVIDYS were vomiting and nausea

- Vomiting occurred in about 65% of children who received ELEVIDYS, and it may occur as early as the day of infusion
- Nausea occurred in about 20% of children who received ELEVIDYS

Side effects typically seen within the first 2 weeks:

- Nausea (41%)
- Vomiting (62%)
- Low platelet count (12%)
- Fever (23%)

Side effects seen within the first 2 months:

- Increased liver enzymes
- Severe immune reaction in the muscles (known as immune-mediated myositis)

Contact your doctor about any questions or concerns about potential side effects

In case of emergency, call 911

Readministration of ELEVIDYS is not recommended.

Your child's immune system may recognize the vector in ELEVIDYS, potentially causing a serious immune response.

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ELEVIDYS treatment reminders

Important reminders after ELEVIDYS infusion

You may find it helpful to print this page and review with your doctor before you leave the treatment center.



ELEVIDYS can increase liver enzyme levels and cause acute serious liver injury. It also can cause a serious immune response in the muscles, called immune-mediated myositis.



Contact your doctor immediately if:

- Your child's skin and/or whites of the eyes appear yellowish as this may be a sign of increased liver enzymes
- Your child experiences any unexpected increased muscle pain, tenderness or weakness, including severe muscle weakness, difficulty swallowing, breathing, or speaking or to weakened vocal cords as this may be a sign of a severe immune reaction in the muscles called immune-mediated myositis



Use proper hand hygiene when coming into direct contact with your child's body waste for 1 month after infusion.



The most common side effects are nausea and vomiting.

Other side effects include low platelet count and fever, in addition to increased liver enzymes and immune-mediated myositis. Report any side effects to your doctor.

Use this space for notes from speaking with your doctor about postinfusion monitoring.

Please see additional [Important Safety Information](#) on page 4 and accompanying full [Prescribing Information](#).

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ELEVIDYS treatment reminders (continued)

After ELEVIDYS infusion, your child will have a specific posttreatment corticosteroid regimen and monitoring plan

You may find it helpful to print this page and use it as a reminder during the posttreatment monitoring period.



Corticosteroid regimen

It's important to follow your doctor's instructions for treatment-related corticosteroid use to help reduce the risk of an immune response to ELEVIDYS. Your child will take this course of corticosteroids for at least 60 days after infusion.

MY CHILD'S DAILY POST-ELEVIDYS CORTICOSTEROID DOSE: _____

- Call your doctor if your child misses a dose or vomits/diarrhea
- Due to corticosteroid use, infections (eg, cold, flu, gas, intestinal issues, ear infection, lung infection, etc) become or after treatment may lead to more serious complications. Call your doctor immediately if you notice any symptoms that are suggestive of infection (eg, cough, runny nose, sore throat, or fever)



Monitoring schedule

Your doctor will monitor your child through blood tests to monitor liver function (weekly for first 3 months), platelet counts (weekly for first 2 weeks), and troponin-I (weekly for first 2 weeks). Your doctor may want to monitor your child for longer.

Use the chart below to write in the date and help track your weekly appointments.

	WEEK 1	WEEK 2	WEEK 3	WEEK 4	WEEK 5
MONTH 1					
MONTH 2					
MONTH 3					

For questions along the way, contact your doctor.

In case of emergency, call 911.

Please see additional Important Safety Information on page 4 and accompanying full Prescribing Information.



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