Elevidys

delandistrogene moxeparvovec-rokl

suspension for intravenous infusion

TREATMENT GUIDE

FOR CAREGIVERS

Please see additional Important Safety Information on pages 4-5 and full Prescribing Information.

Introduction to ELEVIDYS

ELEVIDYS (delandistrogene moxeparvovec-rokl) is a prescription gene therapy used to treat ambulatory and non-ambulatory people with Duchenne muscular dystrophy who are at least 4 years old and have a confirmed mutation in the dystrophin gene.

Use in non-ambulatory people was approved under accelerated approval, which allows for drugs to be approved based on a marker that is considered reasonably likely to predict a clinical benefit. Treatment with ELEVIDYS increased the marker, ELEVIDYS micro-dystrophin (also called "micro-dystrophin"). Verification of a clinical benefit may be needed for ELEVIDYS to continue to be approved for non-ambulatory people with Duchenne.

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:

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 at least 4 years old with Duchenne muscular dystrophy (DMD) who have a confirmed mutation in the *DMD* gene.

ELEVIDYS is approved under accelerated approval for non-ambulatory patients at least 4 years old with DMD who have a confirmed mutation in the *DMD* gene. 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 micro-dystrophin in skeletal muscle. Verification of a clinical benefit may be needed for ELEVIDYS to continue to be approved for non-ambulatory patients with DMD.

Who should not receive ELEVIDYS?

Individuals with certain types of mutations, any deletion in exon 8 and/or exon 9 in the *DMD* gene, should not receive ELEVIDYS.

Please see additional Important Safety Information on pages 4-5 and full Prescribing Information.

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Treatment with ELEVIDYS

There are 5 main steps to treatment with ELEVIDYS

1. **Confirm eligibility**
2. **Blood tests** (to prepare for treatment)
3. **Begin treatment–related corticosteroid regimen** (1 to 7 days before treatment)*
4. **Infusion day**
5. **Weekly safety monitoring** (3+ months) **Gradual corticosteroid dose reduction to baseline** (at least 60 days after infusion)

*Start times range from 1 to 7 days before ELEVIDYS treatment, depending on whether or not your child currently takes corticosteroids.

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

SareptAssist dedicated team members are available.

1-888-SAREPTA (1-888-727-3782)
Monday through Friday, 8:30am – 6:30pm ET

Spanish-speaking team members and interpreters for other languages are also available.

Please see additional Important Safety Information on pages 4-5 and full Prescribing Information.

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Important Safety Information

What is ELEVIDYS?

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Who should not receive ELEVIDYS?

Individuals with certain types of mutations, any deletion in exon 8 and/or exon 9 in the *DMD* gene, should not receive ELEVIDYS.

What is the most important information to know about ELEVIDYS?

Infusion-related reactions, including hypersensitivity and serious allergic reactions (anaphylaxis), have occurred during and after ELEVIDYS infusion. Symptoms may include fast heart rate, fast breathing, swollen lips, shortness of breath, nostrils widening, hives, red and blotchy skin, itchy or inflamed lips, rash, vomiting, nausea, chills, and fever. Your doctor will monitor you during and at least 3 hours after ELEVIDYS infusion. If an infusion-related reaction occurs, your doctor may slow or stop the ELEVIDYS infusion and provide additional medical treatment as needed. Contact a healthcare provider immediately if infusion-related symptoms occur.

ELEVIDYS can increase certain liver enzyme levels and cause acute serious liver injury. Patients will receive oral corticosteroid medication before and after infusion with ELEVIDYS and will undergo weekly blood tests to monitor liver enzyme levels for 3 months after treatment. Contact a healthcare provider immediately if the patient's skin and/or whites of the eyes appear yellowish or if the patient misses a dose of corticosteroid 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 preexisting liver impairment, chronic liver infection, or acute liver disease may be at higher risk of acute serious liver injury.

Immune-mediated myositis (an immune response affecting muscles) was observed in patients with a deletion mutation in the *DMD* gene that is contraindicated. Patients with certain mutation deletions (in exons 1 to 17 and/or exons 59 to 71) may be at risk for a severe immune-mediated myositis reaction. Caregivers should contact a healthcare provider immediately if the patient experiences any unexplained increased muscle pain, tenderness, or weakness, including difficulty swallowing, breathing, or speaking, as these may be symptoms of myositis.

Please see additional Important Safety Information on page 5 and full Prescribing Information.

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Important Safety Information (continued)

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

Myocarditis (inflammation of the heart) has been observed within days following ELEVIDYS infusion. The patient's doctor will conduct weekly blood tests for the first month after treatment to evaluate troponin-I (a cardiac protein that can detect damage to muscle cells in the heart). Caregivers should contact a healthcare provider immediately if the patient begins to experience chest pain and/or shortness of breath. More frequent monitoring may be required if the patient has cardiac symptoms.

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 or prevent desired therapeutic levels. Treatment with ELEVIDYS is not recommended for patients who have high antibodies to the vector, the part of gene therapy used to deliver ELEVIDYS.

Due to the need to follow a corticosteroid regimen, an infection (such as cold, flu, gastroenteritis [stomach flu], otitis media [ear infection], bronchiolitis [respiratory infection], etc) before or after ELEVIDYS infusion could lead to more serious complications. Caregivers should contact a healthcare provider immediately if they see any symptoms suggestive of infection, such as coughing, wheezing, sneezing, runny nose, sore throat, or fever.

Are there any considerations for vaccination schedules and ELEVIDYS?

Patient vaccinations should be up to date with current immunization guidelines. Vaccinations should be received at least 4 weeks prior to starting the corticosteroid regimen that is required before receiving ELEVIDYS.

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

Vector shedding of ELEVIDYS occurs primarily through body waste. Patients and caregivers should use proper hand hygiene, such as hand washing when coming into direct contact with patient body waste. Place potentially contaminated materials that may have the patient's bodily fluids/waste in a sealable bag and dispose into regular trash. Precautions should be followed for 1 month after ELEVIDYS infusion.

What are the possible or likely side effects of ELEVIDYS?

The most common side effects that occurred in patients treated with ELEVIDYS were vomiting, nausea, liver injury, 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).

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

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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:

Age

ELEVIDYS is approved for use in individuals with Duchenne who are at least 4 years of age.

Genetic test

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

ELEVIDYS cannot be used in people with any deletion in exon 8 and/or exon 9 in the *DMD* gene.

Antibody test

Individuals may naturally develop antibodies to the ELEVIDYS vector (AAVrh74). This means that the immune system would recognize the vector, potentially causing a serious immune response after the ELEVIDYS infusion or preventing it from working as intended. If your child's immune system has anti-AAVrh74 antibodies above the required threshold, your child is not eligible for treatment with ELEVIDYS.

Your doctor will perform a blood test to measure the presence of antibodies to the ELEVIDYS vector (AAVrh74). Your doctor will use a specific test, and results will report that antibodies are either "elevated" or "not elevated."

TEST RESULTS:	ELIGIBILITY:
Not elevated	✓ Eligible for ELEVIDYS
Elevated	✕ Not eligible for ELEVIDYS

Enrollment Form

You and your child's doctor must complete and submit a Sarepta Gene Therapy Enrollment Form.

Please see additional Important Safety Information on pages 4-5 and full Prescribing Information.

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Preparing for treatment

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

Vaccination

Your child's immunizations should be up-to-date with current immunization guidelines prior to starting the ELEVIDYS treatment–related corticosteroid regimen required before ELEVIDYS infusion. Vaccinations should be completed at least 4 weeks prior to the initiation of the corticosteroid regimen.

Weight check

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

Preinfusion tests

Your doctor will perform blood tests and a clinical exam before the infusion day. These tests will evaluate liver 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 acute liver disease, your doctor may postpone ELEVIDYS administration until it is resolved or controlled.

Oral corticosteroid regimen

To 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 dose 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 pages 4-5 and 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 ELEVIDYS into a vein in your child's arm.

Duration

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

Your doctor will monitor you during and at least 3 hours after ELEVIDYS infusion. If an infusion-related reaction occurs, your doctor may slow or stop the ELEVIDYS infusion and provide additional medical treatment as needed.

IMPORTANT NOTE:
Tell your doctor about any signs or symptoms of infection before ELEVIDYS administration. If your child has signs of infection, the infusion may need to be postponed due to the increased risk of a serious immune response

Readministration of ELEVIDYS is not recommended.

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

Please see additional Important Safety Information on pages 4-5 and full Prescribing Information.

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After treatment

Postinfusion corticosteroid regimen and tapering

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

Your child should take this dose of corticosteroids for at least 60 days. Your doctor will tell you when to adjust, reduce the dose (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 misses a corticosteroid dose or vomits it up.

Due to corticosteroid use, an infection (eg, cold, flu, stomach flu, ear infection, respiratory infection, etc) before or after ELEVIDYS infusion could lead to more serious complications. Call your doctor immediately if you see symptoms suggestive of infection (eg, coughing, wheezing, sneezing, runny nose, sore throat, or fever).

Postinfusion monitoring

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

- **Liver 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 heart inflammation (weekly for 1 month). More frequent monitoring may be required

See pages 11-12 for ELEVIDYS treatment reminders

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). Practice proper hand hygiene, such as hand washing, when coming into contact with your child's body waste. Place potentially contaminated materials that may have your child's fluids/waste in a sealable bag, and dispose into regular trash.
Follow these precautions for 1 month after ELEVIDYS infusion.

Please see additional Important Safety Information on pages 4-5 and 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 experiences fast heart rate, fast breathing, swollen lips, shortness of breath, nostrils widening, hives, red and blotchy skin, itchy or inflamed lips, rash, vomiting, nausea, chills, or fever as these may be signs of an infusion-related reaction**
- **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 difficulty swallowing, difficulty breathing, or difficulty speaking, as these may be a sign of an immune response affecting muscles (immune-mediated myositis)**
- **Your child experiences chest pain and/or shortness of breath, as these may be a sign of inflammation of the heart (myocarditis)**

The most common side effects across clinical studies were vomiting, nausea, liver injury, fever, and decreased platelet counts.

Side effects following treatment in clinical studies (in more than 5% of participants)

Side effects	ELEVIDYS (N=156) %
Vomiting	65
Nausea	43
Liver injury	40
Fever	28
Decreased platelet counts	8

Side effects following treatment and occurring at least twice more frequently than with placebo* in Study 3 Part 1

Side effects	ELEVIDYS (n=63) %	Placebo (n=62) %
Vomiting	64	19
Nausea	40	13
Liver injury	41	8
Fever	32	24
Decreased platelet counts	3	0

Vomiting may occur as early as on the day of infusion. Side effects typically seen within the first 2 weeks include nausea, vomiting, decreased platelet counts, and fever.

Side effects seen within the first 2 months include an immune response affecting muscles (known as immune-mediated myositis) and liver injury.

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

In case of emergency, call 911

*A placebo is a liquid or pill that has no active medication; you may know it as a sugar pill.

Please see additional Important Safety Information on pages 4-5 and full Prescribing Information.

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ELEVIDYS treatment reminders

Important reminders after ELEVIDYS infusion

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

ELEVIDYS can cause infusion-related reactions, including hypersensitivity and serious allergic reactions (anaphylaxis), increase certain liver enzymes, and cause acute serious liver injury. It can also cause an immune response affecting muscles (immune-mediated myositis) or inflammation of the heart (myocarditis).

Contact your doctor immediately if:

- **Your child experiences fast heart rate, fast breathing, swollen lips, shortness of breath, nostrils widening, hives, red and blotchy skin, itchy or inflamed lips, rash, vomiting, nausea, chills, or fever as these may be signs of an infusion-related reaction**
- **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 difficulty swallowing, difficulty breathing, or difficulty speaking, as these may be a sign of an immune response affecting muscles (immune-mediated myositis)**
- **Your child experiences chest pain and/or shortness of breath, as these may be a sign of inflammation of the heart (myocarditis)**

Call 911 with any life-threatening concerns

Practice proper hand hygiene, such as hand washing, when coming into direct contact with your child's body waste. Place potentially contaminated fluids/waste in a sealable bag, and dispose into regular trash. Follow these precautions for 1 month after infusion.

The most common side effects are vomiting, nausea, liver injury, fever, and decreased platelet counts.

Report any side effects to your doctor.

Use this space for notes from speaking with your doctor about postinfusion monitoring and to record important contact information for easy reference in case of emergency.

TREATING PHYSICIAN PHONE NUMBER

TREATMENT CENTER NAME/TREATING PHYSICIAN NAME

Please see additional Important Safety Information on pages 4-5 and 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 refer to 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 reduce the risk of an immune response to ELEVIDYS. Your child will take this dose 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 it up
- Due to corticosteroid use, an infection (eg, cold, flu, stomach flu, ear infection, respiratory infection, etc) before or after ELEVIDYS infusion could lead to more serious complications. Call your doctor immediately if you see symptoms suggestive of infection (eg, coughing, wheezing, sneezing, runny nose, sore throat, or fever)

Monitoring schedule

Your doctor will order blood tests to monitor:

- Liver function (weekly for the first 3 months)
- Platelet counts (weekly for the first 2 weeks)
- Troponin-I levels (weekly for the first month). More frequent monitoring may be required

You may want to set reminders about these appointments in your calendar or on your phone. Your doctor may want to monitor your child for longer.

For questions along the way, contact your doctor.

In case of emergency, call 911. Tell any hospital, urgent care clinic, or Emergency Department personnel that your child has received ELEVIDYS.

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SAREPTA THERAPEUTICS

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