

Considering ELEVIDYS gene therapy?

START BY TALKING TO YOUR DOCTOR



This discussion guide can help you prepare for a conversation with your doctor about ELEVIDYS.

IMPORTANT SAFETY INFORMATION

What is the most important information to know about ELEVIDYS?

Rapid Serious Liver Injury and Rapid Liver Failure

- **ELEVIDYS can increase certain liver lab test levels and cause rapid serious liver injury, rapid liver failure, and death.** Patients with preexisting liver problems may be at higher risk.
- Complication of blood clots in the blood vessel in the abdomen that helps carry blood from the intestines to the liver has happened.
- Patients will receive oral corticosteroid medication before and after ELEVIDYS infusion and will need weekly blood tests to monitor liver function for 3 months or longer after treatment.
- For at least 2 months following ELEVIDYS infusion, stay close to a healthcare facility that the doctor recommends.
- Contact a doctor 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.

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 not recommended for individuals with:

- Preexisting liver problems or liver infection because of the high risk of rapid serious liver injury and rapid liver failure
- Recent vaccination (within 4 weeks of ELEVIDYS treatment)
- Current or recent infections (within 4 weeks of ELEVIDYS treatment)

Please see additional Important Safety Information on pages 7-8 and full Prescribing Information, including Boxed Warning, and Medication Guide.

 **Elevidys**
delandistrogene
moxeparvovec-rokl
suspension for intravenous infusion

ABOUT THIS GUIDE

The questions that follow have been created in partnership with parents from the Duchenne community and may be a helpful starting place for your next conversation.

All individuals are unique, so please skip or add questions that are applicable to you and your loved ones. You can also print this guide and bring it to your appointment.

To find a treatment center that administers ELEVIDYS, visit [ELEVIDYS.com](https://www.elevidys.com).

ABOUT ELEVIDYS

→ What is the goal of ELEVIDYS gene therapy?

→ How does ELEVIDYS work?

→ What resources are available to support us before, during, and after treatment?

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UNDERSTANDING ELIGIBILITY

→ Who is eligible?

→ Is ELEVIDYS only for specific mutations?

→ What is an antibody test, and how could it impact treatment options?

→ When can I get an antibody test to learn if I'm eligible?

CLINICAL TRIAL RESULTS

→ How much micro-dystrophin does ELEVIDYS produce in the body and what impact might that have?

→ What impact did ELEVIDYS have on motor function?

CLINICAL TRIAL RESULTS *(continued)*

→ How long have people in clinical trials been followed?

→ What results could be expected from treatment?

→ What are the safety considerations of treatment with ELEVIDYS?

→ What side effects might my child experience? When would they be most likely to happen and how long will they last?

→ Can you explain the liver risk warning?

→ Is there any additional information about the potential benefits or risks of ELEVIDYS that I should be aware of?

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THE TREATMENT PROCESS

→ How and where is ELEVIDYS given?

→ What is the treatment process like? How might it impact my loved one(s)?

→ What additional medications are required?

→ How long does the treatment take?

→ What specific precautions do we need to take and how might our daily life be impacted just after treatment?

→ What are the monitoring requirements after treatment, and where does this occur?

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AFTER THE TREATMENT PROCESS

→ What are the next steps for my care plan after receiving ELEVIDYS?

→ After treatment with ELEVIDYS, are other treatments and clinical trial opportunities possible?

ADDITIONAL QUESTIONS I HAVE



We're here to help you, every step of the way.

Wherever you are in the treatment process, whether you're considering if ELEVIDYS is right for your child, preparing for treatment, or navigating posttreatment care, we're here for you.

[Learn more](#) about how our team can help.

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Important Safety Information

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- Patients will receive oral corticosteroid medication before and after ELEVIDYS infusion and will need weekly blood tests to monitor liver function for 3 months or longer after treatment.
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Serious Infection

- Because patients will be taking corticosteroids as part of ELEVIDYS treatment, this may lower the ability of their immune system to fight infections and make it easier to get an infection. Getting an infection (like a cold, flu, stomach flu, ear infection, chest infection) before or after ELEVIDYS infusion could lead to more serious health problems, including death.
- Contact a doctor right away if you notice any signs of infection, such as coughing, wheezing, sneezing, runny nose, sore throat, or fever.
- Vaccinations should be completed at least 4 weeks before starting the corticosteroids that are part of the ELEVIDYS treatment.
- ELEVIDYS should not be given to patients with an infection.

Inflammation of the Heart Muscle

- Serious and life-threatening inflammation of the heart muscle has happened following ELEVIDYS infusion.
- Patients will need weekly blood tests for a heart protein that can detect damage to the heart muscle cells for the first month after treatment.
- Contact a doctor right away if the patient begins to experience chest pain and/or trouble breathing or shortness of breath.

Infusion-related Reactions

- Infusion-related reactions, including hypersensitivity and serious allergic reactions (anaphylaxis), have happened during and after ELEVIDYS infusion.
- Contact a doctor right away if you notice: 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.

Please see additional Important Safety Information on page 8 and full Prescribing Information, including Boxed Warning, and Medication Guide.

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Important Safety Information (continued)

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

Immune Response Affecting Muscles (Immune-mediated Myositis)

- Immune response affecting muscles, including serious and life-threatening reactions, has happened in patients about 1 month after receiving ELEVIDYS infusion.
- Contact a doctor immediately if the patient experiences any unexplained increased muscle pain, tenderness, or weakness, including trouble swallowing, breathing, or speaking.

Antibodies to ELEVIDYS

- Patients need to have blood tests to ensure that they do not have antibodies that may prevent them from being able to receive ELEVIDYS. High levels of antibodies may keep the medicine from working as intended.
- Treatment with ELEVIDYS is not recommended for patients who have high antibodies to the vector, the part of gene therapy used to deliver ELEVIDYS.

You should discuss the potential benefits and risks of ELEVIDYS with your doctor.

Who should not receive ELEVIDYS?

Individuals with a certain type of genetic mutation, called a deletion, involving any portion of or the entire exon 8 and/or exon 9 in the *DMD* gene, should not receive ELEVIDYS.

Are there any considerations for vaccination schedules and ELEVIDYS?

Patient vaccinations should be up to date with current immunization guidelines. Vaccinations should be completed at least 4 weeks before starting corticosteroids that are part of the ELEVIDYS treatment.

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 most common side effects of ELEVIDYS?

The most common side effects that occurred in patients treated with ELEVIDYS were vomiting, nausea, liver injury, fever, lower number of platelets (a kind of blood cell that helps you stop bleeding), and higher levels of heart protein that can detect damage to muscle cells in the heart.

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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