

ELEVIDYS gene therapy for Duchenne muscular dystrophy

ABOUT THE TREATMENT PROCESS



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 4-5 and full Prescribing Information, including Boxed Warning, and Medication Guide.

 **Elevidys**
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An update on _____

As you may know, our son has Duchenne muscular dystrophy, a rare disease that causes muscles in the body to become weak and damaged over time.

After completing the necessary testing to confirm that he is eligible, our child will soon receive a treatment called ELEVIDYS, a prescription gene therapy used to treat ambulatory people with Duchenne muscular dystrophy who are at least 4 years old and have a confirmed mutation in the dystrophin gene.

This is a big decision and an exciting time for our family. As you are an important adult in our child's life, we wanted to share information about what to expect during the ELEVIDYS treatment process.

1 What to know about ELEVIDYS

- ELEVIDYS is a **gene therapy** designed to **provide our child's muscles with genetic instructions** he's missing because of Duchenne
- ELEVIDYS is a **one-time treatment** that enables the body to make a type of protein that may help muscles in people with Duchenne. An infusion is given on 1 day followed by **weekly appointments for at least 3 months for safety monitoring***
- Our son will **receive ELEVIDYS at a special treatment center**. Treatment day will be a big day for our family

TREATMENT DAY: _____ ANTICIPATED END OF WEEKLY APPOINTMENTS*: _____

2 What to know about the ELEVIDYS treatment process

BEFORE ELEVIDYS treatment day, our child will need to:

- ✓ Attend **doctor's appointments** in preparation for treatment
- ✓ Be careful to **avoid getting sick**, as this may delay treatment
- ✓ Practice **proper hand washing** and may take other precautions to stay healthy
- ✓ Take **daily steroid medication** (1 day to 1 week prior to treatment), in addition to current steroid use

DURING the 3-month monitoring period after treatment, our child will need to:

- ✓ Take **daily steroid medication** that is required for ELEVIDYS treatment (these are in addition to current steroid use)
- ✓ Attend **weekly follow-up appointments***
- ✓ Continue practicing **proper hand washing** habits and potentially other precautions to remain healthy

*Our doctor may decide to monitor our son for a longer period of time or more frequently.

Please see additional Important Safety Information on pages 4-5 and full Prescribing Information, including Boxed Warning, and Medication Guide.



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How you can help

This treatment is a commitment for our child and our family. It will mean some missed school days, and we may not be as responsive as usual. Below are a few ways you can help over the next few months:



Support us in staying on track with his medications and appointments.

We will do our best to proactively plan with you around needs and timing.



Reinforce good hygiene with regular hand washing – for both you and our child.



If you notice our child is feeling unwell, please inform us as you normally would. Because our child will be taking daily steroid medication as part of ELEVIDYS treatment, this may lower the ability of the 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.

You can also learn more about potential side effects related to ELEVIDYS and what to look out for in the following Important Safety Information.



Offer extra patience, support, and flexibility.

USE THIS SPACE FOR ANY OTHER NOTES ON HOW TO HELP (meals, carpools, pet care, etc):

We are grateful for your support during this exciting time for our family!



To learn more or access other resources related to ELEVIDYS treatment, go to [ELEVIDYS.com](https://www.elevidys.com)

Please see additional **Important Safety Information** on pages 4-5 and full **Prescribing Information**, including **Boxed Warning**, and **Medication Guide**.

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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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- 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.

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 5 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).

Please see additional Important Safety Information on page 4 and full Prescribing Information, including Boxed Warning, and Medication Guide.

