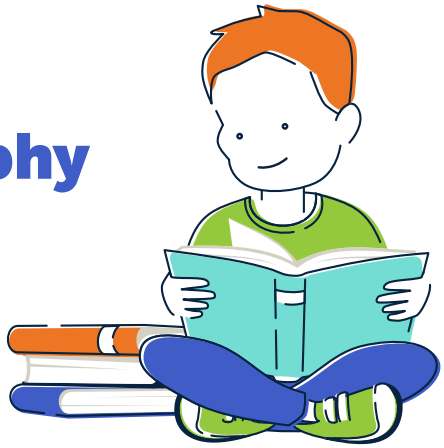
ELEVIDYS gene therapy for Duchenne muscular dystrophy



ABOUT THE TREATMENT PROCESS

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 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.

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*: _____

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

Please see full Indication and Important Safety Information on pages 3-4 and full Prescribing Information.

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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.

3 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

If you notice our child is feeling unwell, please inform us as you normally would. You can learn more about potential side effects related to ELEVIDYS and what to look out for in the following Important Safety Information

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

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

Please see full Indication and Important Safety Information on pages 3-4 and full Prescribing Information.

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Important Safety Information

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.

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 4 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 3 and full Prescribing Information.

SAREPTA THERAPEUTICS

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