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.

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

Use in non-ambulatory people is 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.

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**.

Elevidys
delandistrogene moxeparvovec-rokl
suspension for intravenous infusion

Please see full Indication and Important Safety Information on pages 6-7 and full Prescribing Information.

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?

UNDERSTANDING ELIGIBILITY

→ Who is elegible?

→ Is ELEVIDYS only for specific mutations?

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

Please see full Indication and Important Safety Information on pages 6-7 and full Prescribing Information.

Elevidys
delandistrogene moxeparvovec-rokl
suspension for intravenous infusion

CLINICAL TRIAL RESULTS

→ How was ELEVIDYS studied?

→ How well did ELEVIDYS work in clinical trials?

→ What are the safety considerations of treatment with ELEVIDYS?

THE TREATMENT PROCESS

→ How and where is ELEVIDYS given?

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

→ What additional steroids are required?

Please see full Indication and Important Safety Information on pages 6-7 and full Prescribing Information.

Elevidys
delandistrogene moxeparvovec-rokl
suspension for intravenous infusion

THE TREATMENT PROCESS *(continued)*

→ 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 side effects could we experience? How long might they last, and what can be done to manage them?

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

Please see full Indication and Important Safety Information on pages 6-7 and full Prescribing Information.

Elevidys
delandistrogene moxeparvovec-rokl
suspension for intravenous infusion

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

SAREPTASSIST

For more information about ELEVIDYS, visit **ELEVIDYS.com** or contact SareptAssist at 1-888-SAREPTA (1-888-727-3782).

Case Managers are available Monday through Friday, 8:30am – 6:30pm ET

Please see full Indication and Important Safety Information on pages 6-7 and full Prescribing Information.

Elevidys
delandistrogene moxeparvovec-rokl
suspension for intravenous infusion

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 7 and full Prescribing Information.

Elevidys
delandistrogene moxeparvovec-rokl
suspension for intravenous infusion

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 6 and full Prescribing Information.

SAREPTA THERAPEUTICS

Elevidys
delandistrogene moxeparvovec-rokl
suspension for intravenous infusion