January 26, 2026

Community Letter: Update on the 3-Year EMBARK Trial Data that Shows Disease-Modifying Impact of Our Approved Gene Therapy in Treatment of Duchenne

Dear Duchenne community,
Today, topline results of three-year data from individuals treated in Part 1 of the global Phase 3 EMBARK clinical trial (NCT05096221) were announced.
Also today, at 4 p.m. ET, PPMD will host a community webinar to discuss results, and we hope many of you can join to learn more. Registration link here.

The EMBARK trial investigated treatment with ELEVIDYS (delandistrogene moxeparvovec-rokl) in ambulatory individuals with Duchenne who were aged 4-7 years old at the beginning of the study. Upon completion of EMBARK, participants were eligible to enroll in a follow-up study to observe long-term efficacy and safety study (EXPEDITION, NCT05967351). Thus, participants who were dosed in the initial (Part 1) treatment group of EMBARK and enrolled in EXPEDITION (n=52) have up to 3 years of post-gene therapy follow-up. Analysis of data for these patients show:

- **Positive Results Across All Endpoints:** Statistically significant and clinically meaningful efficacy was seen across all key functional outcomes - the North Star Ambulatory Assessment (NSAA), Time to Rise (TTR), and 10-meter walk/run (10MWR) - compared to a well-matched, external control group. Results confirm the efficacy outcomes seen at the end of year 2.
- **Continued Treatment Effect:** The 3-year data show a slower progression of the disease and a widening of the difference between boys treated with ELEVIDYS and those in the external control group treated with corticosteroids.
- **Consistent Safety Profile:** No new safety signals were observed. The safety profile of the gene therapy remains consistent and manageable based on treatment of more than 1,200 people in clinical trial and real-world settings, including more than 1,050 ambulatory people.
- Full 3-year EMBARK data will be presented at an upcoming medical conferences and publications.
- **Continued Safety Monitoring:** Study participants continue to be followed for at least five years after treatment via the EXPEDITION study.

We deeply appreciate the partnership and commitment of the Duchenne community, your families and the advocates who support you.

Should you have questions or need more information, please reach out to us at advocacy@sarepta.com.
Sincerely,

Wendy Erler

Senior Vice President, Patient Affairs

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.

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.

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.

Indication

What is ELEVIDYS (delandistrogene moxeparvovec-rokl)?

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)

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 the full Prescribing Information for ELEVIDYS, including Boxed Warning and Medication Guide.


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