November 16, 2025

Dear Duchenne community,

Recently, we shared the announcement that the prescribing information (also known as "the label") for ELEVIDYS (delandistrogene moxeparvovec-rokl), the only approved gene therapy for Duchenne muscular dystrophy (DMD) has been updated. With this letter, we want to share what has changed, provide the MedicationGuide, answer questions about what it means for families and patients interested in pursuing treatment, and reaffirm our commitment to ensuring patient safety, which is our highest priority.

What is a label change?

The prescribing information (PI) label is a living document that provides instructions and essential scientific information to healthcare professionals for the safe and effective use of a medicine. Often times, especially in rare disease, as real-world experience with a prescribed product grows post-approval, updates to the label are made to reflect new learnings around patients' experiences with the therapy, including those related to adverse events.

Sarepta initiated the labeling update process for ELEVIDYS following the passing of a non-ambulatory patient from acute liver failure following treatment with ELEVIDYS, and has been working with FDA over the last several months to make updates to the labeling.

The revised labeling can be accessed PILINK as well as the MedicationGuide.

What changed with the ELEVIDYS label?

As previously shared, we voluntarily suspended commercial shipments of ELEVIDYS for non-ambulatory patients in June, while we pursued plans for an additional study of an enhanced immunosuppressive regimen in non-ambulatory patients.

At this time, the FDA review of the updated label is complete. Here we share key updates to the ELEVIDYS label:

- A boxed warning for the risk of acute serious liver injury (ALI) and acute liver failure (ALF). A boxed warning is meant by FDA to highlight certain contraindications or serious warnings and to call prominent attention to healthcare providers about potential adverse reactions and safe use instructions.
- Removal of language regarding the use of ELEVIDYS for non-ambulatory patients. These

measures aim to ensure the safe administration of ELEVIDYS in ambulatory patients while equipping healthcare providers, patients, and families with the scientific information needed to support informed, individualized treatment discussions.

- Expanded guidance for prescribers, including a modified pre- and post-infusion oral corticosteroids regimen and enhanced monitoring recommendations on a weekly basis for 3 months post-infusion to help ensure patients receiving ELEVIDYS have a standardized pre- and post-infusion treatment and monitoring experience.
- A new warning in the Warnings & Precautions section regarding increased susceptibility for serious infections due to immunosuppression.

Our commitment to non-ambulatory Duchenne patients remains unwavering. This label update allows us the time to study the use of enhanced immunosuppression therapy in non-ambulatory patients such that we will be able to have a more informed, evidence-based discussion regarding the path to future access to ELEVIDYS by non-ambulatory patients.

We understand the urgency this group of patients and their families feel; we feel it, too. We will work diligently with regulators and healthcare professionals and, most importantly, you, the Duchenne community, to ensure that non-ambulatory patients have equal access to informed decision-making about their care and the benefit-risk profile of innovative treatment options and will continue to support the entire Duchenne community.

We encourage families to read the Medication Guide and to talk to their doctor for a full understanding of the risks and benefits on an individual patient level.

Sincerely,

Wendy Erler
Senior Vice President, Patient Affairs