



**U.S. FOOD & DRUG**  
ADMINISTRATION

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## Memorandum

**DATE:** May 23, 2023

**TO:** Rachel Duddy, MS, RPM, CBER/OTAT/DRPM  
Emmanuel Adu-Gyamfi, Ph.D., Committee Chair, CBER/OTAT/DCGT  
Mike A. Singer, M.D., Ph.D., Clinical Reviewer, CBER/OTAT/DCEPT

**FROM:** Benjamin S. Cyge, Ph.D.  
Consumer Safety Officer  
APLB/DCM/OCBQ

**THROUGH:** Lisa L. Stockbridge, Ph.D.  
Branch Chief  
APLB/DCM/OCBQ

**SUBJECT: ELEVIDYS (delandistrogene moxeparvovec-rokl)**  
**BLA: 125781/0**  
Sponsor: Sarepta Therapeutics, Inc.

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## Background

The sponsor submitted:

☒ New Approval  
☐ Changes Being Effectuated (CBE) supplement  
☐ Prior Approval Supplement (PAS)  
☐ Major Amendment

Submission contains:

☒ Prescribing Information (PI)  
☐ Patient Package Insert (PPI)  
☒ Package and/or container labels  
☐ Other

Submission Date: September 28, 2022

PDUFA Action Date: June 22, 2023

## **APLB Comments/Recommendations**

This is a labeling review for BLA 125781, submitted by Sarepta Therapeutics, Inc. for ELEVIDYS (delandistrogene moxeparvovec-rokl) on September 28, 2022. ELEVIDYS is an adeno-associated virus vector-based gene therapy indicated for the treatment of ambulatory pediatric patients, aged 4 to 5 years, with Duchenne muscular dystrophy (DMD) with a confirmed mutation in the *DMD* gene.

APLB reviewed the draft prescribing information (PI), package, and container labels dated September 28, 2022. The following comments are from a promotional and comprehension perspective.

### **GENERAL**

- Replace the placeholder “xxxx” with the approved suffix, “rokl,” throughout the PI.
- Avoid the use of bolding unless it is required by regulation.
- Avoid language that is promotional in tone.

### **FULL PRESCRIBING INFORMATION**

#### **DOSAGE AND ADMINISTRATION**

There are multiple areas throughout this section where bolding is used when it is not required by regulation. For example, in steps e, f, and g in subsection 2.2, and in the subheading “Monitoring Post-Administration.” These statements can be capitalized and/or italicized for emphasis, but not bolded.

#### **ADVERSE REACTIONS**

Include a list of the most frequently occurring adverse reactions, along with the criteria used to determine inclusion (e.g., incidence rate greater than x%). Ensure this statement is consistent with the ADVERSE REACTIONS section in the HIGHLIGHTS.

#### **CLINICAL PHARMACOLOGY**

Avoid the use of language that is promotional in tone. Only statements of objective facts should be included.

#### **PATIENT COUNSELING INFORMATION**

This section has poor readability, due to being too dense and having mixed concepts in a paragraph. This section is intended to provide clinicians with key information needed to be easily shared with patients and their caregivers. Please change the format of this section into a parallel, bulleted list of specific concepts. Begin with *Inform Caregivers*, followed with the bullets of the things the clinician must talk about with the caregiver.

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## **PACKAGE AND CONTAINER LABELS**

Replace the suffix placeholder “xxxx” with the proper approved suffix, “rokl.”

If you have any questions regarding this review, please contact Benjamin S. Cyge, Consumer Safety Officer at (301) 796-4212.