

MEMORANDUM

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Acting Director, Office of Therapeutic Products

FDA / CBER / OTP

BLA STN 125781 / 0

Submission received date: September 28, 2022

Applicant: Sarepta Therapeutics, Inc.

Product: Proper / Proprietary Name: delandistrogene moxeparvovec-rokl / ELEVIDYS

Proposed indication: For treatment of ambulatory patients with Duchenne muscular dystrophy (DMD) with a confirmed mutation in the *DMD* gene

Summary

Sarepta Therapeutics, Inc. (the Applicant) submitted Biologics License Application (BLA) 125781 to seek Accelerated Approval for delandistrogene moxeparvovec-rokl, with the proprietary name of ELEVIDYS, based on the proposed surrogate endpoint of expression of ELEVIDYS micro-dystrophin at Week 12 following administration of ELEVIDYS. The purpose of this memo is to provide my perspective regarding the regulatory action (i.e., Accelerated Approval vs. a Complete Response (CR)) for this BLA.

The clinical review team and the related disciplines (i.e., clinical pharmacology and statistical) recommend a CR for this BLA. This recommendation was overridden by the CBER Center Director, Dr. Peter Marks. Please refer to Dr. Marks' memo for additional information. It will be the regulatory decision of the Center Director to grant BLA 125781 Accelerated Approval for the treatment of ambulatory pediatric patients aged 4 through 5 years with Duchenne muscular dystrophy (DMD) with a confirmed mutation in the *DMD* gene.

The memo from the clinical review team concludes that available data do not support use of ELEVIDYS micro-dystrophin expression as a surrogate endpoint "reasonably likely to predict clinical benefit" for Accelerated Approval of ELEVIDYS for the treatment of ambulatory patients with DMD with a confirmed mutation in the *DMD* gene or certain subpopulation(s) of ambulatory patients with DMD with a confirmed mutation in the *DMD* gene. I agree with this conclusion. Please see the review documents and memos in BLA 125781 administrative record for details.