



U.S. FOOD & DRUG
ADMINISTRATION

Memorandum

DATE April 27, 2023

FROM Triet M. Tran, PharmD, Consumer Safety Officer
Bioresearch Monitoring Branch (BMB)
Division of Inspections and Surveillance (DIS)
Office of Compliance and Biologics Quality (OCBQ)
Telephone: 240-425-3201

THROUGH Dennis T. Cato, Chief BMB

THROUGH Carrie M. Mampilly, MPH, Director DIS

TO Emmanuel Adu-Gyamfi, PhD, Chair, STN 125781/0
Xiaofei Wang, PhD, Clinical Reviewer
Rachel Duddy, MS, RPM

SUBJECT Bioresearch Monitoring Final Review Memo
SPONSOR Sarepta Therapeutics, Inc.
PRODUCT ELEVIDYS (Delandistrogene moxeparvovec (SRP-9001))
BLA STN 125781/0

Final Summary Statement

Bioresearch Monitoring (BIMO) inspections assignments were issued for two domestic clinical investigator (CI) sites participating in the conduct of study Protocol SRP-9001-102 and SRP-9001-103. The inspections did not reveal significant problems impacting the data submitted in support of this Biologics License Application (BLA).

Background

BIMO inspection assignments were issued for two CI sites. One site participated in the conduct of study Protocols SRP-9001-102 and SRP-9001-103, while the other site participated solely in the conduct of Protocol SRP-9001-103. The BLA review committee concurred with the sites selected for inspection. The sites were selected based upon sponsor-reported adverse events, deaths, protocol deviations, total number of enrolled subjects and previous BIMO inspection histories. The inspection assignments were issued for the following study protocols:

Protocol SRP-9001-102 - A Randomized, Double-blind, Placebo-controlled Study of SRP-9001 (Delandistrogene Moxeparvovec) for Duchenne Muscular Dystrophy (DMD)

Protocol SRP-9001-103 - An Open-Label, Systemic Gene Delivery Study Using Commercial Process Material to Evaluate the Safety of and Expression From SRP-9001 in Subjects with Duchenne Muscular Dystrophy (ENDEAVOR)

BIMO CI inspections were conducted in accordance with FDA's Compliance Program (CP) 7348.811, Inspection Program for CI. One of the two sites participated solely in study SRP-9001-103, while the other participated in both study SRP-9001-102 and SRP-9001-103. The inspections represented 90% of the study population in study SRP-9001-102 and 56% of the study population in study SRP-9001-103. The inspection assignments included specific questions related to the study protocols, and information submitted in the BLA was compared to source documents at each inspected site.

The inspections verified the data reported in the BLA, including but not limited to subject eligibility, protocol deviations, study drug administration, primary efficacy endpoint, and adverse events for all subjects enrolled at the inspected clinical sites.

Inspection Outcome

No significant objectionable inspectional findings were observed during the inspection. The table below summarizes the BIMO inspections:

Site ID	Number of subjects randomized	Location	483 Issued	Final Inspection Classification
201	14 (<u>SRP-9001-102</u>) 37 (<u>SRP-9001-103</u>)	Jerry R. Mendell MD Columbus, OH	No	No Action Indicated (NAI)
210	8 (<u>SRP-9001-103</u>)	Craig Zaidman MD St. Louis, MO	No	NAI

Noteworthy inspectional findings

The inspections did not reveal substantive issues that impact the data submitted in the BLA.

Sponsor Issues

No significant sponsor issues were noted.

Clinical Investigator Issues

The inspections verified the data reported in the BLA, including but not limited to subject eligibility, protocol deviations, study drug administration, primary efficacy endpoint, and adverse events for all subjects enrolled at the inspected clinical sites. No Form FDA 483s were issued for any of the three inspected study sites.

Financial Disclosure

The CI CP directs the FDA investigators to ask the CI if and when he/she disclosed information about his/her financial interests to the sponsor and/or interests of any sub-investigators, spouses and dependent children, and if and when the information was last updated. The information submitted to the BLA was verified at the inspected sites no deviations were found in the submitted data.

Administrative follow-up

No administrative follow-up is warranted at this time. Should you have any questions about the contents of this memo or any aspect of BIMO, please contact me at 240-425-3201.

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