



U.S. FOOD & DRUG
ADMINISTRATION

DATE: June 20, 2024

FROM: Haecin Chun, MS, Consumer Safety Officer
Bioresearch Monitoring Branch (BMB)
Division of Inspections and Surveillance (DIS)
Office of Compliance and Biologics Quality (OCBQ)

THROUGH: Dennis T. Cato, Associate Director, Bioresearch Monitoring (BIMO)

THROUGH: Carrie M. Mampilly, MPH, Director, DIS

TO: Bao-Ngoc Nguyen, PhD, Chair, BLA 125807/0
Chinwe Okoro, MD, Clinical Reviewer
Hawa Camara, MS, PMP, RPM

SUBJECT: BIMO Final Discipline Review Memo

SPONSOR: Abeona Therapeutics, Inc.
PRODUCT: Prademagene zamikeracel
Application: BLA 125807/0

FINAL SUMMARY STATEMENT

BIMO inspection assignments were issued for the sponsor and two clinical investigator (CI) study sites that participated in the conduct of Protocol EB-101-CL-301 (VIITAL). The inspections did not reveal significant issues that impacted the data submitted in this original Biologics License Application (BLA).

BACKGROUND

A sponsor and two Clinical Investigator (CI) BIMO inspection assignments were issued in support of this original BLA. The inspection reviewed the study conduct of the protocol entitled, "*A Phase 3, Study of EB-101 for the Treatment of Recessive Dystrophic Epidermolysis Bullosa (RDEB)*" (Protocol EB-101-CL-301 (VIITAL)).

These inspections were conducted in accordance with FDA's Compliance Program (CP) 7348.810, Inspection Program for Sponsors and Contract Research Organizations and CP 7348.811, Inspection Program for CIs. Information and data submitted in the BLA were compared to source documents at each inspected site, and the inspection assignments included specific questions concerning the clinical study.

INSPECTION SUMMARY AND OUTCOME

The table below summarizes the BIMO inspections:

Type	Site ID	Study Site Name and Location	483 Issued?	Status
Sponsor	N/A	Abeona Therapeutics Inc. Cleveland, OH	No	No Action Indicated (NAI)
CI	01	Stanford University, School of Medicine Redwood City, CA	No	NAI
CI	02	UMass Medical School Clinical Research Center Worcester, MA	No	NAI

Sponsor:

No significant sponsor issues were observed during the inspection.

Clinical Investigators:

No significant issues were observed during the CI inspections; however, the inspection revealed some minor issues that did not substantively impact the safety of subjects and/or the data submitted in support of the BLA. The following minor discrepancies between the source data and data submitted in the BLA:

Site 01: There were a few minor transcription errors of adverse events for four subjects and one missing hypo-albuminemia was missing from the FDA data listing. The clinical team was notified on 4/5/2024.

Site 02: Five (5) data entry errors related to an incorrect pain scale scoring were observed during the inspection. One (1) entry had an incorrect year for the date. The clinical team was notified on 3/7/2024.

FINANCIAL DISCLOSURE:

The CI CP directs the FDA investigator 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, spouse(s) and dependent children, and if and when the information was last updated. The information submitted to the BLA was verified for each of the inspected clinical sites.

ADMINISTRATIVE FOLLOW-UP:

Should you have any questions or comments about the contents of this memo or any aspect of Bioresearch Monitoring, please contact me at haecin.chun@fda.hhs.gov.

Haecin Chun
Consumer Safety Officer