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## Memorandum

DATE March 26, 2026

FROM Jennifer Chan, PharmD, Consumer Safety Officer  
Bioresearch Monitoring Branch (BMB)  
Division of Inspections and Surveillance (DIS)  
Office of Compliance and Biologics Quality (OCBQ)

THROUGH Kanaeko R. Sharp, MS, Chief BMB

THROUGH Carrie M. Mampilly, MPH, Director DIS

TO Bo Liang, PhD, Chair  
Ning Hu, MD, MS, Clinical Reviewer  
Prateek Shukla, MD, Clinical Reviewer  
Rachel Blasdel, RPM  
Helen Sansone, RPM

SUBJECT Bioresearch Monitoring Final Discipline Review Memo  
SPONSOR Regeneron Pharmaceuticals, Inc.  
PRODUCT Otarmeni (lunsotogene parvec-cwha)  
STN BLA 125874/0

### FINAL SUMMARY STATEMENT

Bioresearch Monitoring (BIMO) inspections were issued for one foreign and two domestic clinical investigators (CIs) participating in the conduct of study Protocol DB-OTO-001. The inspections did not reveal significant issues impacting the data submitted in support of this original Biologics License Application (BLA).

### BACKGROUND

BIMO inspection assignments were issued for one foreign and two domestic CIs that participated in the study conduct of study Protocol DB-OTO-001. The sites were selected based on previous inspectional history, geographic location, and the data submitted in the BLA. The BLA review committee concurred with the sites selected for inspection. These inspections were conducted in accordance with FDA's Compliance Program (CP) 7348.811, Inspection Program for Clinical Investigators.

### PROTOCOL

*Protocol DB-OTO-001: A Phase 1/2, Open-Label, Multicenter Trial with a Single Ascending Dose Cohort with Unilateral Intracochlear Injection Followed by a Bilateral Injection Expansion Cohort to Evaluate the Safety, Tolerability, and Efficacy of DB-OTO in Children and Infants with Biallelic hOTOF Mutations*

The inspection assignment included specific questions related to the study protocol and information submitted in the BLA was compared to source documents at the clinical sites.

Study DB-OTO-001 was conducted at 11 sites across the United States (US), Spain, the United Kingdom (UK), and Germany, enrolling a total of 24 subjects at the time of the last data cutoff. The three CIs inspected in support of this BLA covered approximately 46% of the total study population enrolled in the study.

#### INSPECTION SUMMARY AND OUTCOME

The inspections verified the data reported in the BLA, including but not limited to subject eligibility, study drug administration, protocol deviations, and adverse events for the reviewed subjects enrolled at the inspected clinical site. No significant objectionable inspectional findings were observed during the inspections. The table below summarizes the BIMO inspections:

Site ID	Firm Name	Location	Form FDA 483 Issued?	Final Inspection Classification
ES005	Dr. Ruben Polo Lopez, MD	Madrid, Spain	No	No Action Indicated (NAI)
US008	Lawrence Lustig, MD	New York, NY	No	NAI
US010	Steven Harvey, MD	Milwaukee, WI	No	NAI

#### Inspectional Findings:

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

#### Sponsor Issues:

No significant sponsor issues were noted.

#### 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.

#### 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 Jennifer Chan at (301) 348-1897.

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Jennifer Chan, PharmD.  
Consumer Safety Officer

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