



U.S. FOOD & DRUG
ADMINISTRATION

DATE: October 29, 2024

FROM: Peter Lenahan, DC, PhD, MPH
Bioresearch Monitoring Branch (BMB)
Division of Inspections and Surveillance (DIS)
Office of Compliance and Biologics Quality (OCBQ)

THROUGH: Kanaeko Ravenell, MS, Branch Chief, BMB

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

TO: CBER Connect STN 125722/0
Chair: Bo Liang, PhD
Clinical: Avanti Golikeri, MD
Tolani Inhola, PharmD, RPM

SUBJECT: Bioresearch Monitoring Final Discipline Review Memo
SPONSOR: PTC Therapeutics, inc.
PRODUCT: Eladocagene exuparvovec
BLA/STN 125722/0

Final BIMO SUMMARY STATEMENT

Bioresearch Monitoring (BIMO) inspection assignments were issued for one foreign clinical investigator (CI) who participated in the conduct of the clinical trial PTC-AADC-GT-002 and NTUH-AADC-011; and one domestic clinical investigator who participated in PTC-AADC-GT-002. A third inspection assignment was issued to one foreign clinical investigator who participated in Protocol PTC-AADC-011. The inspections did not reveal substantive issues that impact the data submitted in this supplemental Biologics License Application (sBLA).

BACKGROUND

BIMO inspection assignments were issued for one foreign clinical investigator (CI) who participated in the conduct of the clinical trial PTC-AADC-GT-002 and NTUH-AADC-011; and one domestic clinical investigator who participated in PTC-AADC-GT-002. A third inspection assignment was issued to one foreign clinical investigator who participated in Protocol PTC-AADC-011. The BLA clinical review committee concurred with the proposed sites. The sites were selected based upon sponsor-reported adverse events, subject deaths, protocol deviations, total numbers of enrolled subjects, previous BIMO inspection histories, BLA clinical review team recommendations, and CI financial disclosures.

The inspections were conducted in accordance with FDA's Compliance Program (CP) 7348.811, Inspection Program for CIs. Information submitted in the sBLA was compared to source documents at each inspected site. The inspection assignment also included specific questions concerning the clinical study.

Protocol Number: PTC-AADC-GT-002: (b) (4)

According to the clinical study report, a total of six (6) study centers in three (3) countries (USA, Taiwan, and Israel) enrolled a total of 13 subjects into this pivotal study.

Protocol NTUH-AADC-010: A Phase I/II clinical trial for treatment of aromatic L-amino acid decarboxylase (AADC) deficiency using AA V2-hAADC

Protocol NTUH-AADC-011: A clinical trial for treatment of aromatic L-amino acid decarboxylase (AADC) deficiency using AA V2-hAADC - An expansion

BIMO INSPECTIONS SUMMARY

At close of the inspections no significant BIMO inspectional findings were noted, no Forms FDA-483 were issued, and all inspections were classified No Action Indicated (NAI).

The below table summarizes site information and outcomes from the BIMO inspections.

Site Number	Location	Form FDA-483 Issued	Final Classification
101	Houston, TX	No	NAI
No Site ID Issued (AADC-011)	Taipei, Taiwan	No	NAI
002 (GT-002) & 011 (AADC-011)	Taipei, Taiwan	No	NAI

SIGNIFICANT INSPECTIONAL FINDINGS

No significant inspectional findings or significant data integrity issues were observed.

SPONSOR/MONITORING ISSUES

No significant sponsor monitoring issues were identified during the above inspections.

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, as well as if and when the information was last updated. The information submitted to the sBLA was verified for each of the inspected clinical study sites.

ADMINISTRATIVE FOLLOW-UP

Should you have any questions or comments about the contents of this memo or any aspect of BIMO, please contact me at (301) 837-7156.

Respectfully,

Peter Lenahan, DC, PhD, MPH
CDR, U.S. Public Health Service
Senior Regulatory Reviewer

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