



**U.S. FOOD & DRUG**  
ADMINISTRATION

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DATE: October 17, 2024

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

THROUGH: Kanaeko R. Ravenell, MS, Chief BMB

THROUGH: Carrie M. Mampilly, MPH, Director DIS

TO: Crystal Melendez, MT, RN, BSN, DCPM, RPM, BLA 125798/0  
Panico Carolina, MD, PhD, Chair,  
Tsilou Ekaterini, MD, Clinical Reviewer

SUBJECT: Bioresearch Monitoring Final Discipline Review

SPONSOR: Neurotech Pharmaceuticals, Inc,

PRODUCT: Revakinagene taroretcel (NT-501)

BLA: 125798/0

**FINAL SUMMARY STATEMENT:**

Bioresearch Monitoring (BIMO) inspection assignments were issued for one foreign and two domestic clinical investigators (CI) that participated in the conduct of Protocols NTMT-03-A and NTMT-03-B. The inspections did not reveal substantive issues that impact the data submitted in this Biologics License Applications (BLA).

**BACKGROUND:**

Three BIMO CI inspection assignments were issued in support of this BLA. The sites were selected based upon geographic location, reported adverse events, protocol deviations, number of subjects enrolled, and previous BIMO inspection histories. The BLA review committee concurred with the proposed sites.

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

**PROTOCOL:**

Protocol NTMT-03: A Phase 3 Multicenter, Randomized, Sham-Controlled Study to Determine the Safety and Efficacy of NT-501 in Macular Telangiectasia Type 2

**BIMO INSPECTIONS SUMMARY:**

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

<b>Site ID</b>	<b>Firm Name</b>	<b>Location</b>	<b>FDA Form 483 Issued</b>	<b>Final Classification</b>
04	Alain Gaudric, MD	Paris, France	No	No Action Indicated (NAI)
10	Lawrence Singerman, MD	Cleveland, OH	No	NAI
50	Amani Fawzi, MD	Chicago, IL	No	Voluntary Action Indicated (VAI)

**INSPECTIONAL FINDINGS:**

There were no significant observations for Study Sites 04 and 10. However, the following observations were noted for Study Site 50:

**Study Site 50:**

- Three subjects were consented with the wrong version of the informed consent form.
- For one subject, an Adverse Event (AE) was not reported and several others inconsistently documented. Eighteen (18) AEs were recorded on the AE Log but only fourteen (14) were recorded on the individual AE forms.
- Documentation of the submission of digital eye images were not retained for all visits for all enrolled subjects.
- For some subjects, the enrollment dates on source document worksheets were not consistent with the dates eligibility was confirmed.

The corrective action plans presented from the CI have been determined to be adequate and acceptable by BIMO.

**SPONSOR MONITORING ISSUES:**

No significant sponsor or 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 BLA was verified for each of the inspected clinical study sites.

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ADMINISTRATIVE FOLLOW-UP

Post inspection correspondence was issued for the inspected parties.

Should you have any questions or comments about the contents of this memo or any aspect of Bioresearch Monitoring, please contact me at (240) 402-0767.

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Yakubu Wangabi  
Consumer Safety Officer