



Memorandum

DATE August 13, 2025

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 Laura DeMaster, PhD, Chair
Cherie Fathy, MD, Clinical Reviewer
Gumei Liu, MD, Clinical Reviewer
Cecilia Crowley, RPM

SUBJECT Bioresearch Monitoring Final Discipline Review Memo
SPONSOR Fondazione Telethon ETS (FTE)
PRODUCT WASKYRA (Autologous CD34+ Cells Transduced with Lentiviral
Vector Expressing Human Wiskott-Aldrich Syndrome Gene; OTL-103)
STN BLA 125846/0

FINAL SUMMARY STATEMENT

Bioresearch Monitoring (BIMO) inspections were issued for the sponsor and one foreign clinical investigator (CI) site participating in the conduct of study Protocols 201228 and OTL-103-4. The inspections did not reveal significant issues impacting the data submitted in support of this Biologics License Application (BLA).

BACKGROUND

BIMO inspection assignments were issued for the sponsor and one foreign CI site that participated in the study conduct of study Protocols 201228 and OTL-103-4. 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) 7348.810, Inspection Program for Sponsors and Contract Research Organizations and CP 7348.811, Inspection Program for Clinical Investigators.

PROTOCOLS

Protocol 201228: *A phase I/II clinical trial of hematopoietic stem cell gene therapy for the Wiskott-Aldrich syndrome (TIGET-WAS)*

Protocol OTL-103-4: A Single Arm, Open Label Clinical Study of Haematopoietic Stem Cell Gene Therapy with Cryopreserved Autologous CD34+ Cells Transduced with Lentiviral Vector encoding WAS cDNA in Subjects with Wiskott-Aldrich Syndrome (WAS)

The inspection assignment included specific questions related to the study protocols, and information submitted in the BLA was compared to source documents at the clinical site. Study 201228 was conducted at one site in Italy, which enrolled a total of 8 subjects. Study OTL-103-4 was conducted at two sites across the United States and Italy, enrolling a total of 10 subjects. The one CI site inspected in support of this BLA covered 100% and 90% of the total study population enrolled in the 201228 and OTL-103-4, respectively.

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	Study Site Name and Location	Form FDA 483 Issued?	Final Inspection Classification
04	Ospedale San Raffaele – Telethon Institute for Gene Therapy (SR-TIGET) Milan, Italy 20132	No	No Action Indicated (NAI)
Sponsor	Fondazione Telethon ETS (FTE) Milan, Italy 20129	No	NAI

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.

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.

Jennifer Chan, PharmD.
Consumer Safety Officer

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