



U.S. FOOD & DRUG
ADMINISTRATION

Memorandum

DATE: October 4, 2023

TO: Nobuko Katagiri, PhD, Chair
Megha Kaushal, MD, Clinical Reviewer
Cara Pardon, MS, RPM

FROM: Malcolm Nasirah, PharmD, MS, Regulatory Reviewer
Bioresearch Monitoring Branch (BMB)
Division of Inspections and Surveillance (DIS)
Office of Compliance and Biologics Quality (OCBQ)

THROUGH: Dennis T. Cato, Chief BMB

THROUGH: Carrie M. Mampilly, MPH, Director DIS

SUBJECT: Bioresearch Monitoring Discipline Review Memo

PRODUCT: recombinant ADAMTS13 (TAK-755)

SPONSOR: Takeda Pharmaceuticals USA, Inc
BLA STN: 125795/0

REVIEW SUMMARY

Bioresearch Monitoring (BIMO) Clinical Investigator (CI) inspection assignments were issued for one foreign and three domestic clinical study sites that participated in the conduct of protocol TAK-755-3002. The completed inspections did not reveal substantive issues that impact the data submitted in this Biologics License Application (BLA).

BACKGROUND

Four clinical study sites conducting the study protocol TAK-755-3002 were identified for BIMO CI inspections. The sites were selected based upon the inspectional history, sponsor-reported adverse events, protocol deviations, and total number of subjects enrolled.

The inspections were conducted in accordance with FDA's Compliance Program (CP) 7348.811, Inspection Program for CIs. Information submitted in the BLA was compared to source documents at each inspected site. The inspection assignment also included specific questions concerning the clinical study protocol TAK-755-3002.

PROTOCOL

TAK-755-3002: A Phase 3b, prospective, open-label, multicenter, single treatment arm, continuation study of the safety and efficacy of TAK-755 (rADAMTS-13, also known as BAX 930/SHP655) in the prophylactic and on-demand treatment of subjects with severe congenital thrombotic thrombocytopenic purpura (cTTP; Upshaw-Schulman Syndrome, or hereditary thrombotic thrombocytopenic purpura)

BIMO INSPECTIONS SUMMARY

No significant BIMO inspectional findings were noted for the inspections. The below table summarizes site information and outcomes from the BIMO inspections.

Study Site #	Firm Name	Location	FDA Form 483 Issued	Inspectional Final Classification
24	Ana Antun, MD	Atlanta, GA	No	NAI – No Action Indicated
26	Thomas Ortel, MD	Durham, NC	No	NAI
28	Sami Ibrahimi, MD	Oklahoma City, OK	No	NAI
18	Marie Ann Scully	United Kingdom	No	NAI

INSPECTIONAL FINDINGS:

There were no significant observations at any of the inspected clinical study sites, and a Form FDA 483 was not issued for any of the inspections.

SPONSOR/MONITORING ISSUES

No significant sponsor or monitoring issues were identified during the completed 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.

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 301-796-6667.

Malcolm Nasirah, PharmD, MS, BCGP
Consumer Safety Officer

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