



U.S. FOOD & DRUG
ADMINISTRATION

DATE: July 20, 2026

FROM: Char-Dell Edwards Consumer Safety Officer
Bioresearch Monitoring Branch (BMB)
Division of Inspections and Surveillance (DIS)
Office of Compliance and Biologics Quality (OCBQ)

THROUGH: Kanaeko Sharp, MS, Branch Chief BMB

THROUGH: Carrie M. Mampilly, MPH, Director DIS

TO: Jacob Bitterman, PhD, Committee Chair
Jemila Venner-Walcott, MD, Clinical Reviewer
Yakun Wang, PhD, Biostats Reviewer
Tolani Ishola, PharmD, RPM

SUBJECT: Bioresearch Monitoring Final Discipline Review
SPONSOR: Ultragenyx Pharmaceutical
PRODUCT: GENGLYCOS (pariglasgene breCAParvovec (DTX401, AAV8GSDIa))
BLA STN: 125858/0

FINAL SUMMARY STATEMENT:

Bioresearch Monitoring (BIMO) inspection assignments were issued for one foreign and two domestic clinical investigator (CI) study sites who participated in the conduct of study protocol DTX401-CL301. The inspections did not reveal substantive issues that impact the data submitted in this Biologics License Application (BLA).

BACKGROUND:

Three CI study sites (two domestic and one foreign) for protocol DTX401-CL301 were identified for BIMO inspections. The BLA review committee concurred with the proposed sites. The sites were selected based upon sponsor-reported adverse events, protocol deviations, number of subjects enrolled, and previous BIMO inspection histories.

The inspections were conducted in accordance with FDA's Compliance Program (CP) 7348.811: Inspection Program for Clinical Investigators and Sponsor-Investigators. 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:

Protocol DTX401-CL301: A Phase 3, randomized, double-blind, placebo-controlled study of Adeno-Associated Virus (AAV) Serotype 8 (AAV8)-Mediated Gene Transfer of Glucose-6-Phosphatase (G6Pase) in Patients with Glycogen Storage Disease Type Ia.

BIMO INSPECTIONS SUMMARY:

No significant BIMO inspectional observations were identified. The table below summarizes site information and outcomes from the BIMO inspections.

Site ID	Firm Name	Location	Form FDA 483 Issued?	Final Classification
102	Areeg Hassan El-Gharbawy, MD, DSc	Durham, North Carolina	No	NAI (No Action Indicated)
112	Margo Sheck Breilyn, MD, MS	New York, New York	No	NAI
201	John James Mitchell, FRCPC, MD, MSc	Montreal, Quebec, Canada	No	NAI

INSPECTIONAL FINDINGS:

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

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.

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 (240) 402-2859.

Char-Dell K. Edwards, BS, MT(ASCP)
Consumer Safety Officer