



U.S. FOOD & DRUG
ADMINISTRATION

Memorandum

DATE January 9, 2024

FROM Triet M. Tran, PharmD, BCSCP, Regulatory Officer
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

TO Anna Kwilas, PhD, Chair
Kavita Natrajan, MD, Clinical Reviewer
Umer (Muhammad) Choudhry, MD, Clinical Reviewer
Danielle Bauman, RPM

SUBJECT Bioresearch Monitoring Final Review Memo
SPONSOR Vertex Pharmaceuticals Inc.
PRODUCT Exa-cel (Exagamglogene autotemcel)
BLA STN 125785/0

The clinical study sites participating in the conduct of the pivotal study supporting this application have a robust inspection history, and the review team determined that Bioresearch Monitoring (BIMO) inspections are not warranted to support the review of this BLA.

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 - 9689.

Triet M. Tran, PharmD, BCSCP.
Regulatory Officer

Electronic Copies

EDR BLA STN 125785/0

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