

DBSQC/OCBQ ANALYTICAL METHOD REVIEW MEMO

To: The file STN 125846/0

From: Wei Tu, MD
Laboratory of Biochemistry, Virology, and Immunochemistry (LBVI)
Division of Biological Standards and Quality Control (DBSQC)
Office of Compliance and Biologics Quality (OCBQ)
Center for Biologics Evaluation and Research (CBER)
Food and Drug Administration (FDA)

Through: Muhammad Shahabuddin, Ph.D.
Lab Chief, LBVI/DBSQC/OCBQ/CBER/FDA

Maryna Eichelberger, Ph.D.
Division Director, DBSQC/OCBQ

Applicant: Fondazione Telethon ETS/FTS

Subject: Review of Analytical Methods used for WASKYRA (etuvetidigene autotemcel) Lentiviral Vector (LVV) lot release

Recommendation: Approval

Executive Summary:

The following analytical methods used for lot release of WASKYRA and the associated analytical method validations or qualifications, were reviewed:

(b) (4)

Conclusion:

The analytical methods for measuring (b) (4), and their respective platform validations, were reviewed and found to be adequate for their intended use in the WASKYRA LVV. To ensure the specific product's safety profile has been adequately addressed, the reviewer

has requested a product-specific validation, the sponsor has committed to delivering product-specific validation protocols and reports by March 31, 2026.

Documents Reviewed:

Information in sections of the original submission that describe DS (b) (4) (3.2.S), including control of DS and manufacture were reviewed. Additional information in amendment 16, amendment 19, amendment 20, and amendment 22, were also reviewed.

Background:

On January 10, 2025, Fondazione Telethon ETS submitted Biological License Application (STN 125846) for WASKYRA (etuvetidigene autotemcel). WASKYRA consists of autologous CD34+ hematopoietic stem and progenitor cells (HSPC) transduced with a recombinant replication-incompetent lentiviral vector (LVV) encoding the WAS cDNA sequence. The manufacturing process involves producing the WAS LVV (b) (4)

Since the manufacturing process can introduce impurities such as (b) (4) that may cause adverse toxic or immunologic reactions, multiple analytical methods including (b) (4) assays are employed to monitor and quantify impurity levels in (b) (4) drug product to ensure product quality, efficacy, and safety.

(b) (4)

5 pages have been determined to be not releasable: (b)(4)