



MEMORANDUM

Date: November 07, 2024

From: George Kastanis, MS
Quality Assurance Branch (QAB)
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)

To: The file: STN 125722/0

Subject: Review of Lot Release Protocol (LRP) Template for Eladocogene exuparvovec included in STN 125722/0

Through: Varsha Garnepudi, MS, Branch Chief QAB/DBSQC/OCBQ/FDA
Maryna Eichelberger, PhD, Director, DBSQC/OCBQ/CBER/FDA

Applicant: PTC Therapeutics, Inc.

Product: Eladocogene exuparvovec
Trade Name: KEBILIDI

Summary: The LRP template for Eladocogene exuparvovec submitted in amendment 125722/0.62 on October 31, 2024, is acceptable for use.

1 General Information

1.1 CMC Review Identifiers and Dates

1.1.1 Biologics License Application STN: 125722/0

1.1.2 Submission received by CBER: March 15, 2024

1.1.3 Review completed: November 07, 2024

1.1.4 Material Reviewed: BLA 125722/0

1.1.5 Related Master File, INDs and BLAs: STN 125722/0

2 Review

2.1 Documents Reviewed

The LRP template for Eladocagene exuparvovec submitted on March 15, 2024, in BLA 125722/0

The LRP template for Eladocagene exuparvovec submitted on October 15, 2024, in amendment 125722/0.53

The LRP template for Eladocagene exuparvovec submitted on October 31, 2024, in amendment 125722/0.62

2.2 Review

PTC Therapeutics, Inc. submitted an LRP template on March 15, 2024 with their application. The LRP template was reviewed by OTP/OGT and OCBQ/DBSQC with comments regarding acceptance criteria for in-process tests and to add additional information for the (b) (4) acceptance criteria. An IR was sent to PTC Therapeutics, Inc. on October 09, 2024.

A response was submitted on October 15, 2024, in amendment 125722/0.53. This revised LRP template was reviewed by OCBQ/DBSQC and OCBQ/DMPQ/PRB with comments regarding formatting and to add CBER templates for (b) (4) assays. Other comments included to add "result and specification" below the Sterility CBER template and to revise the (b) (4) table to include (b) (4). An IR was sent to PTC Therapeutics, Inc. on October 28, 2024.

A response was submitted on October 31, 2024, in amendment 125722/0.62. The revised LRP template was reviewed by OCBQ/DBSQC and OCBQ/DMPQ/PRB with no comments.

As an aside, it should be noted that in the application, the sponsor did not mention that they would (b) (4) samples prior to testing. The (b) (4) test samples was reviewed by Yen Phan as part of the LRP template review, as sample (b) (4) was not mentioned in their method validation report. The (b) (4) of the samples with the (b) (4) was found acceptable for lot release.

3 Conclusions

The lot release protocol template for Eladocagene exuparvovec submitted in amendment 125722/0.62 on October 31, 2024, is acceptable for use. This template may be used for future lot release submissions.