

DBSQ/OCBQ ANALYTICAL METHOD REVIEW MEMO

To: The file STN 125722

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Applicant: PTC Therapeutics, Inc

Subject: Review of Analytical Methods used for KEBILIDI (Eladocagene exuparvovec) (b) (4)
[redacted] Lot Release

Recommendation: Approval

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

(b) (4) [redacted]
[redacted]
[redacted]
[redacted]
[redacted]
[redacted]
[redacted]
[redacted]

Conclusion:

The analytical methods and their validations and qualifications reviewed for the KEBILIDI (b) (4) [redacted] were found to be adequate for their intended use.

Documents Reviewed

Information in sections of the original submission that describe control of (b) (4) (3.2.S.4), including descriptions of (b) (4) specifications, analytical procedures and validation of these analytical procedures were reviewed. Additional information in amendment 16, amendment 29, amendment 51, and amendment 58, were also reviewed.

Background:

On March 15, 2024, PTC Therapeutics submitted a Biologics License Application (STN125722) for KEBILIDI, a treatment of aromatic L-amino acid decarboxylase (AADC) deficiency. AADC deficiency is an ultra-rare autosomal recessive disorder caused by mutations in the DDC gene, which encodes the AADC enzyme. It typically appears in young children and leads to a complete halt in motor development. KEBILIDI is a recombinant non-replicating adeno-associated virus serotype 2 (rAAV2) vector comprising a dopa decarboxylase variant 2 cDNA transcript, which encodes human AADC (b) (4) , under the control of the (b) (4)

(b) (4) human embryonic kidney (b) (4) (b) (4) cells with a

Monitoring and controlling the levels of the impurities are crucial to ensure the quality and safety of the final adeno-associated virus (AAV) product for patient administration. Several analytical methods, including (b) (4) (b) (4) , are used to monitor and quantify the (b) (4) impurities.

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