



Food & Drug Administration
10903 New Hampshire Ave.
Silver Spring, MD 20993

DBSQC/OCBQ ANALYTICAL METHOD REVIEW MEMO

To: Biologics License Application, STN 125722/0

From: Hsiaoling Wang, Ph.D., LAC/DBSQC/OCBQ

Through: James Kenney, D.Sc. for Maryna Eichelberger, Ph.D.
Acting Division Director, DBSQC/OCBQ

Sponsor: PTC Therapeutics, Inc.

Subject: Review of lot-release test methods for Eladocogene exuparvec drug substance, drug product and in-process control

Recommendation: Approval

Executive Summary:

Analytical methods used for lot release of Eladocogene exuparvec were reviewed by Hsiaoling Wang (LAC), Yen Phan (LMIVTS), Noel Bachoo and Wei Tu (LBVI). Their review memoranda are attached to this cover letter.

Conclusion: The analytical methods and their validations and/or qualifications reviewed for the Eladocogene exuparvec drug substance, drug product and in-process control were found to be adequate for their intended use, except for the outstanding issues for (b) (4) drug product. PTC Therapeutics has provided written commitment to resolve the issues as Postmarketing Study Commitment (PMC) in amendment 50. An additional PMC is committed in amendment 55 to assess the suitability of the drug product sterility method to detect (b) (4), an environmental isolate, to provide additional sterility assurance.