

DBSQ/OCBQ ANALYTICAL METHOD REVIEW MEMO

To: BLA STN 125798/0

From: Jing Lin, Ph.D., RAC
Laboratory of Biochemistry, Virology, and Immunochemistry (LBVI)
Division of Biological Standards and Quality Control (DBSQ)
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/DBSQ/OCBQ/CBER

Maryna Eichelberger, Ph.D.
Division Director, DBSQ/OCBQ/CBER

Product: NT-501 (Encelto) (Implant containing allogeneic retinal pigment epithelial cells expressing recombinant human ciliary neurotrophic factor)

Applicant: Neurotech Pharmaceuticals, Inc.

Subject: Review of Analytical Methods used for NT-501 (b) (4) Drug Product (DP), and Lot Release

Recommendation: Approval

Executive Summary:

The following analytical method used for lot release of NT-501 DP was reviewed:

1. (b) (4) in NT-501

Conclusion: The analytical method reviewed for the NT-501 DP was found to be adequate for its intended use.

Documents Reviewed:

Information in sections of the original submission that describe control of (b) (4) DP (3.2.S.4, and 3.2.P.5, respectively), including descriptions of (b) (4) DP specifications, analytical procedures of (b) (4) DP and validation of these analytical procedures were reviewed. In addition, responses to Information Request (IR) received on August 12, 2024, in amendment 125798/0.21 as well as responses to IR received on November 27, 2024, in amendment 125798/0.68 were also reviewed.

Background:

On April 18, 2024, Neurotech Pharmaceuticals, Inc. submitted an original Biological License Application (BLA) STN 125798/0 for a biologic-device combination product, NT-501(Encelto) (Implant containing allogeneic retinal pigment epithelial [RPE] cells expressing recombinant human ciliary neurotrophic factor [rhCNTF]) for the treatment of macular telangiectasia type 2 [MacTel], a neurodegenerative retinal disease that causes central vision deterioration.

The (b) (4)

(b) (4). The drug product (DP) is 200,000 to 440,000 formulated NTC-201-6A cells encapsulated in a NT-501 implant, a semi-permeable pre-assembled capsule (PAC). Each PAC consists of a semipermeable (b) (4) hollow fiber membrane (HFM) containing an internal scaffold of (b) (4) of polyethylene terephthalate (PET) scaffold yarn, (b) (4) methacrylate (b) (4) adhesive (b) (4) that seals the semi-permeable capsule at each end, and a titanium anchor loop (fixation loop) attached to one end of the semi-permeable capsule which is used to facilitate placement and retrieval of NT-501. The overall dimension of the capsule is approximately 6.5 mm long, with a maximum external diameter of (b) (4) mm.

NT-501 is surgically implanted into the posterior chamber of the eye during an outpatient surgical procedure.

Review:

1. (b) (4) in NT-501


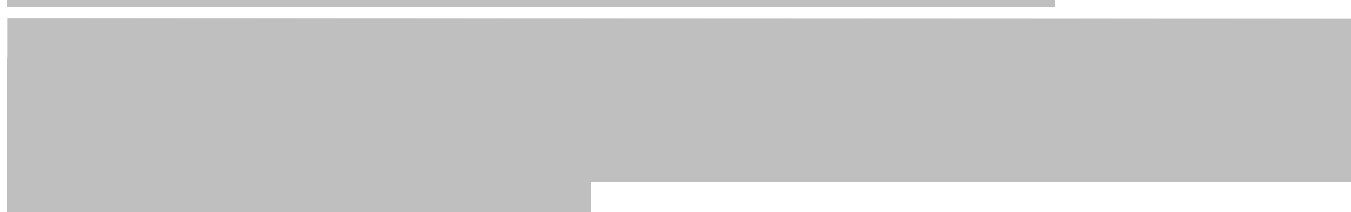


Introduction

One NT-501 implant is a single-use encapsulated cell therapy (ECT) that contains 200,000 to 440,000 NTC-201-6A cells. The method, (b) (4) in NT-501, is intended to be used as lot release and stability assay for NT-501. The release test is performed by Neurotech Pharmaceuticals, Inc.

Method

(b) (4)

(b) (4)



Conclusion

The method of (b) (4) in NT-501 was well described and the validation test results complied with the predefined acceptance criteria. Therefore, the method is appropriately validated and is suitable for its intended use.