



DBSQC/OCBQ ANALYTICAL METHOD REVIEW MEMO

To: The file STN 125798/0

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Sponsor: Neurotech Pharmaceuticals, Inc.

Subject: Review of Analytical Methods used for NT-501 (Implant containing allogeneic retinal pigment epithelial cells expressing recombinant human ciliary neurotrophic factor [rhCNTF] for the treatment of macular telangiectasia type 2 [MacTel]) drug product (DP) Lot Release.

Recommendation: Approval

Executive Summary:

The manufacturing of NT-501 is a continuous process. The following analytical methods used for lot release of NT-501 DP and the associated analytical method validations or qualifications, were reviewed.

1. pH of DP
2. Appearance of DP

Conclusion: The analytical methods and their validations and/or qualifications reviewed for the NT-501 DP were found to be adequate for their intended use.

Documents reviewed:

Information in sections of the original submission that describe (b) (4) DP (3.2.S.4 and 3.2.P.5, respectively), including descriptions of (b) (4) DP specifications, lot release testing of DP and validation of these analytical procedures were reviewed.

Background

On April 24, 2024, Neurotech Pharmaceuticals (NP), Inc. submitted a new Biologics License Application (BLA), STN125798 for the implant NT-501 encapsulated cell technology (ECT). The implant NT-501 ECT consists of allogeneic retinal pigment epithelial cells transfected with plasmid vector (b) (4) to express recombinant human ciliary neurotrophic factor [rhCNTF], encapsulated in a hollow fiber membrane (HFM) in endothelial serum free medium (Endo-SFM). The cells encapsulated in the semipermeable hollow-fiber membrane secrete therapeutic doses

of ciliary neurotrophic factor (CNTF) into the back of the eye for the treatment of retinal degenerative diseases such as Idiopathic MacTel type 2, a bilateral degenerative condition of the macula that may cause progressive loss of vision.

Review Narrative

1. pH – DP

Introduction

The pH specification of NT-501 DP manufactured at Neurotech Pharmaceuticals (NP) Cumberland is (b) (4)

Method

pH determination of NT-501 DP is performed as per document number (b) (4) based on the (b) (4)

(b) (4)

(b) (4)

(b) (4)

Conclusion

As a standard (b) (4) procedure this method is suitable for measuring pH of DP at NP Cumberland, Rhode Island.

2. Appearance - DP

Introduction

The appearance testing for the NT-501 DP is conducted by visual inspection (b) (4)

(b) (4) The DP release specifications are 1) physical state of NT-501 is solid with metallic loop on one end and cap on the other end; Hold medium is liquid and may contain visible particles. 2) white to off white color; Hold medium is orange to pink. 3) clarity of DP is opaque, hold medium is clear. The lot release test for appearance is performed at the NP facility in Cumberland, RI.

Method:

The appearance test is a (b) (4) following SOP-2176 for evaluating the visual physical state of DP and hold medium with respect to foreign particulates, color, and clarity. Appearance tests of hold medium are performed in the (b) (4)

Method Validation

(b) (4)

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Conclusion:

The information provided to support validation of the appearance test at the NP facility, Cumberland, RI is adequate. The method is suitable for lot release testing at this facility.