



DBSQ/OCBQ ANALYTICAL METHOD REVIEW MEMO

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Applicant Ultragenyx Pharmaceutical Inc (Ultragenyx)

Subject Review of (b) (4), endotoxin, and sterility analytical methods used for GENGLYCOS™ (pariglasgene breccaparovect, DTX401, AAV8GSDIa)

Recommendation: Approval

Executive Summary

The (b) (4), endotoxin, and sterility analytical methods used for testing and release of DTX401, and the associated analytic method qualifications were reviewed. The assays were adequately described and shown to be suitable for their intended purpose.

Conclusion

The analytical methods and their qualifications reviewed for DTX401 drug substance and drug product were found to be adequate for their intended use.

Documents Reviewed

Information in sections of the original submission that describe control of Drug Substance (DS) and Drug Product (DP) (3.2.S.4 and 3.2.P.5, respectively), including descriptions of DS and DP specifications, analytical procedures of DS and DP, and qualifications of these analytical procedures, were reviewed. In addition, the responses to CBER's Information Requests (IRs) received on February 25, 2026 (Amendment # 5) were also reviewed as mentioned below.

Background

On December 23, 2025, Ultragenyx submitted the final part of their rolling Biologics License Application (BLA) for DTX401, an adeno-associated virus serotype 8 (AAV8)-based gene therapy. The product is a concentrated solution for intravenous infusion containing human G6PC gene coding sequence encapsidated within a recombinant

AAV8 vector particle. Each DP vial is aseptically filled to a target volume of approximately (b) (4) mL to allow for an extractable volume of 2.0 mL. This review will cover the microbiological assays performed on DTX401 DS and DP to determine the suitability of the test methods for their intended use.

1. (b) (4)

(b) (4)

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

(b) (4)



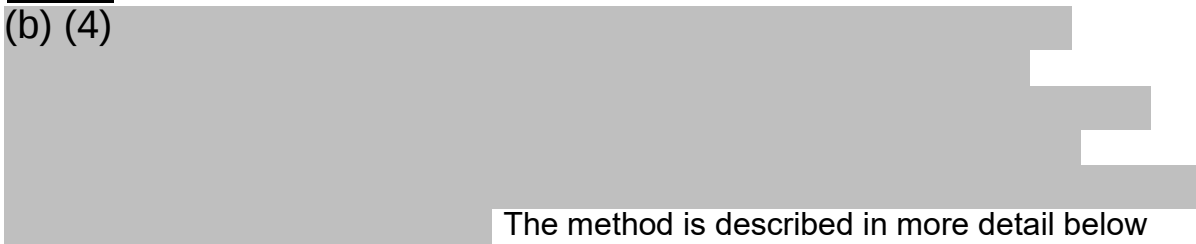
3. Endotoxin Method (b) (4) DP)

Introduction

Endotoxin testing for (b) (4) DP is performed at Ultragenyx in Woburn, MA. Specification of (b) (4) must be met for release of DTX401 (b) (4) DP.

Method

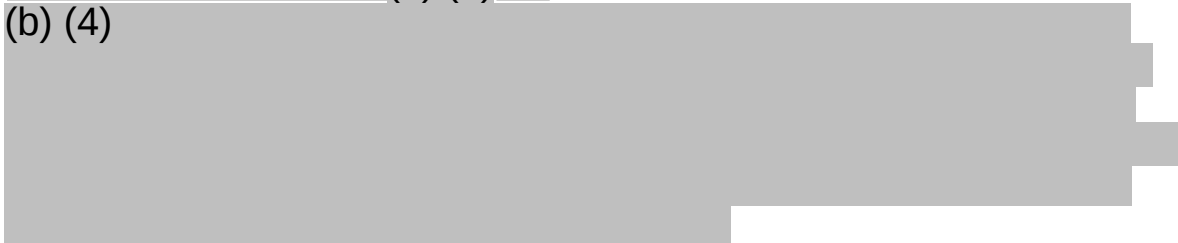
(b) (4)



The method is described in more detail below together with the tests performed to determine the suitability of the test method for its intended use.

Endotoxin Qualification for (b) (4) DP

(b) (4)



(b) (4)

(b) (4)

Conclusion

The method suitability tests were performed and compliant with (b) (4) thus indicating the (b) (4) is appropriate under the actual conditions of use for (b) (4) DP.

4. Sterility Method (DP)

Introduction

Sterility testing for DP is performed at (b) (4). Specification of 'No Growth' must be met for release of DTX401 DP.

Method

The (b) (4) sterility test is used in accordance with (b) (4)

(b) (4)

. The method is described in more detail below together with the tests performed to determine the suitability of the test method for its intended use.

Sterility Qualification for DP

(b) (4)

(b) (4)

CBER found their response acceptable.

Conclusion

The method suitability tests were performed and compliant with (b) (4) thus indicating the (b) (4) sterility test method is appropriate under the actual conditions of use for DP.