Department of Health and Human Services
Public Health Service
Food and Drug Administration
Center for Biologics Evaluation and Research

To:	To File (BLA STN 125062/674)
From:	Nancy Eller, CSO, PDB/DPPT/OTAT Nancy L. Eller -S Digitally signed by Nancy L. Eller -S DN: c=US, o=U.S. Government, ou=HHS, ou=FDA, ou=People, cn=Nancy L. Eller -S, 0.9.2342.19200300.100.1.1=13001445B9 Date: 2021.06.22 18:26:34 -04'00'
Through:	Dr. Dorothy Scott, MD, Laboratory Chief, PDB/DPPT/OTAT Dorothy E. Scott -S Digitally signed by Dorothy E. Scott -S DN: c=US, o=U.S. Government, ou=HHS, ou=FDA, ou=People, 0.9.2342.19200300.100.1.1=13000831 23, cn=Dorothy E. Scott -S Date: 2021.06.22 22:37:51 -04'00'
CC:	Adriane Fisher, Regulatory Health Project Manager, DRPM/OTAT/DRPM/RPMBI
Applicant:	OCTAPHARMA Pharmazeutika Produktionsges.m.b.H
Product:	Immune Globulin Intravenous, Human 10% S/D Trade name: Octagam 10%
Subject:	Efficacy Supplement – New Indication for Dermatomyositis CMC – review of (b) (4) assay

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Recommendation

The recommendation is for approval with the following PMC.

Post-Marketing Commitment

1. Octapharma commits to completing the validation of the (b) (4) assay at the Octapharma Vienna (OPG) and Octapharma Stockholm (OAB) sites, and to setting a final release specification following the testing of (b) (4) of Octagam 10%. Octapharma commits to submitting this as a Prior Approval Supplement (PAS) by June 30, 2022.

Background Summary

In March 2016 Octapharma submitted an IND for Octagam 10% for a Prospective, Double-blind, Randomized, Placebo-Controlled Phase III Study Evaluating Efficacy and Safety of Octagam 10% in Patients with Dermatomyositis. (IND # 016925). The initial clinical hold letter for IND 016925 dated May 23, 2016 contained question 15 b) referring to the development of (b) (4) assay to measure (b) (4) Octagam 10%:

(b) (4)

An assay was developed by Octapharma and presented to FDA in a type C meeting (CRTMS # 11647). Preliminary responses were received by Octapharma on March 28, 2019. The overall proposal for use of

the (b) (4) assay as (b) (4) assay as a release test was found to be acceptable.

Supplement Review Summary

Related Submissions

1. IND 016925 – The study for dermatomyositis was initiated on February 27, 2017 and completed on November 5, 2019.
2. CRTMS # 11647 – Type C Meeting

Documents Reviewed

1. 3.2.P.2 - Pharmaceutical Development
2. (b) (4)
3. (b) (4)
4. 1.14.1.2 – Annotated Draft Labeling Text

Manufacturing Sites Affected

1. Octapharma AB (= OAB)
   Lars F orssells gata 23
   SE - 1 12 75, Stockholm
   FEI: 30055599 15

2. Octapharma Pharmazeutika Produktionsges.m.b.H (= OPG)
   Oberlaaer Strasse 235
   1 100 Vienna, Austria
   FEI: 3002809097

Overview

Octapharma submitted this Efficacy Supplement to add a new indication, dermatomyositis, for the product Octagam 10%. Octapharma proposed the use of (b) (4) assay as an (b) (4) assay (b) (4) (b) (4) This is reflected by the (b) (4) (b) (4) treating dermatomyositis.

Assay Design

(b) (4)

(b) (4)

(b) (4)

Reviewer's Comments – The use of (b) (4)

(b) (4) (b) (4), (b) (5)

(b) (4), (b) (5)

(b) (4), (b) (5)

Validation

The following characteristics were addressed:

(b) (4)

Table 1 – Validation Results

Evaluated parameters	Acceptance criteria	Results	Evaluation
(b) (4)			

(b) (4)

(b) (4)

The (b) (4) were measured within every sequence. Octapharma (b) (4) and the results are shown below in Table 2.

Table 2 – (b) (4)

Evaluated parameters	Acceptance criteria	Results	Evaluation
(b) (4)			

(b) (4)

Table 3 – (b) (4)

(b) (4)

Table 4 – (b) (4)

(b) (4)

(b) (4)

Table 5 – (b) (4)

(b) (4)

(b) (4)

(b) (4)

(b) (4)

Reviewer Comments – Octapharma (b) (4)

(b) (4)

(b) (4) *Octapharma* (b) (4) *will submit a PAS with the fully validated assay and set a final specification.*

(b) (4)

Figure 1: (b) (4)

(b) (4)

(b) (4)

Appendix A

4.4.4 ***Assay sequence***