

From: Jennifer Wellman
To: Glen, Jacqueline
Cc: Morris, Nevitt; Paul Gil
Subject: Re: Information request for BLA 126610
Date: Saturday, June 03, 2017 1:30:30 PM
Attachments: image001.png

image002.png

(File Attachment comment)

(File Attachment comment)

Dear Ms. Glen,

I acknowledge receipt of this email and have included my colleague, Paul Gil, Ph.D., Regulatory CMC Lead, on this reply. Thank you, Jennifer

Jennifer A. Wellman

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From: "Glen, Jacqueline" <Jacqueline.Glen@fda.hhs.gov>
Date: Friday, June 2, 2017 at 4:40 PM
To: Jennifer Wellman <Jennifer.Wellman@sparktx.com>
Cc: "Morris, Nevitt" <Nevitt.Morris@fda.hhs.gov>
Subject: Information request for BLA 126610

Dear Ms. Wellman:

Please provide a response to the information requested by June 9, 2017.

CMC Information Request (June 1, 2017):

1.

In your submission of "2.3.S Quality Overall Summary" Table 8 "AAV2 Manufacturing Lots Included in Process Database used for Development of Interim Control Strategy", you listed 9 lots manufactured at CHOP and 4 lots

manufactured at Spark. Please provide a chart including the following information for each lot listed in the Table 8:

a.

Date manufactured and released

b.

Lot scale

c.

Purpose and the usage of each lot such as:

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- i.
Clinical use
- ii.
Manufacturing process development and qualification
- iii. Comparability study
- iv. Stability study
- v.
Generate the testing specifications for lot release or for comparability studies

2.
Please provide a summary table including all of the lot release testing results for the product lots listed in your Table 8 "AAV2 Manufacturing Lots Included in Process Database used for Development of Interim Control Strategy". Please include all available testing results of drug substances and drug products.

3.
Please provide detailed facility information for the "Secondary Packaging" for your final product (one vial of Drug Product is co-packaged with two vials Diluent). Please provide the detailed information for the packaging facility and process qualification for the "Secondary Packaging". Please include the shipping conditions and validation reports, including, storage conditions and package labeling process qualification information.

4.
Please clarify whether your Drug Product vial and Diluent vial will be labeled at (b) (4) or at the "Secondary Packaging" facility. Please provide the detailed procedures for labeling the vials, packages, boxes, and for the shipping.

5.
Regarding the delivery devices listed in your "Surgical Training Manual for the Administration of LUXTURNA". Please clarify if any of the listed in Tables 1, 2, and 3 devices (i.e., cannulas, extension tubes, and syringes) are 510(k)-cleared or exempt. Please provide the 510(k) numbers for those devices if applicable.

6.
Please provide data that supports compatibility of the product (Luxturna) with all delivery device components (e.g., cannulas, syringes and extension tubes) that are listed in the "Surgical Training Manual", and that will be in contact with the product during formulation in the pharmacy and during subretinal administration in the patient.

The BLA submission contains two Biocompatibility Reports in Section 3.2R that contain data to support compatibility of the product with the delivery system (Study Report TR2016-046 and Study Report TR2016-049). However, we note that the following device components listed in the "Surgical Training Manual" are not covered by either study report:

- 1). Cannula: Retinal hydrodissection cannula 20 g/39 g (Storz Ophthalmic - Bausch & Lomb)
- 2). Syringe: Medallion® 1-mL syringe (Merit Medical Systems, Inc)

Please provide the missing information/data to support product compatibility with all device components.

If you have any questions, please contact Nevitt Morris at 240-402-8269 or Nevitt.Morris@fda.hhs.gov.

Thank you
Jackie

Jacqueline Glen, MS
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