

From: Paul Gil
To: Morris, Nevitt
Cc: Bi, Lilia Lei
Subject: RE: BLA 125610 CMC/ PMC Information Request 12.01.17
Date: Sunday, December 03, 2017 1:59:37 PM
Attachments: image002.png

(File Attachment comment)
Hi Nevitt,
I acknowledge receipt of this email.
Thanks,
Paul

From: Morris, Nevitt [mailto:Nevitt.Morris@fda.hhs.gov]
Sent: Friday, December 1, 2017 9:51 PM
To: Jim Wang <jim.wang@sparktx.com>; Paul Gil <Paul.gil@sparktx.com>
Cc: Morris, Nevitt <Nevitt.Morris@fda.hhs.gov>; Bi, Lilia Lei <LiliaLei.Bi@fda.hhs.gov>
Subject: BLA 125610 CMC/ PMC Information Request 12.01.17

Hi Jim and Paul:

Below is our proposed final wording for the CMC PMCs. If you agree to these PMCs as written, please copy and submit to the BLA. If you have any questions on the wording of the PMCs, please request a telecon to discuss with FDA on Dec. 4, 2017.

1.
Spark Therapeutics, Inc. commits to provide the shipping validation study protocol for shipment of the Drug Product from the distributor to a clinical site (or to Spark Therapeutics, Inc.) by January 31, 2018. A final study report will be submitted as a "Postmarketing Commitment - Final Study Report" by June 30, 2018.

2.
Spark Therapeutics, Inc. commits to complete the verification studies for the following assays:

a.

(b) (4)

b.

(b) (4) tests for particulate matter for the Drug Product and Diluent, performed by (b) (4).

A final study report will be submitted as a "Postmarketing Commitment - Final Study Report" by March 31, 2018.

3.

Spark Therapeutics, Inc. commits to perform an analysis of the lot release test

results obtained from all Drug Substance (DS) and Drug Product (DP) lots manufactured within the first (b) (4) following approval, and evaluate if the

acceptance criteria for LUXTURNA lot release tests (including the

(b) (4)

) continue to provide adequate quality control for DS and DP based on the new data obtained from those tests.

A final study report will be submitted as a "Postmarketing Commitment - Final

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Study Report" by March 31, 2020.

4.

Spark Therapeutics, Inc. commits to conduct stability studies on the HEK293

Master Cell Bank (MCB) used for drug substance manufacture. (b) (4)

, "Postmarketing Commitment - Final Study Report" by March 31, 2018.

5.

Spark Therapeutics, Inc. commits to qualify the (b) (4)

. A

final

study report will be submitted as a "Postmarketing Commitment - Final Study

Report" by March 31, 2018.

Thanks and please acknowledge receipt of this email.

Nevitt

Nevitt Morris

Nevitt

Morris,

RN,

BSN,

BS

Consumer

Safety

Officer

Office

of

Tissues

and

Advanced

Therapies

Center

for

Biologics

Evaluation

and

Research

(CBER)

U.S.

Food

and

Drug

Administration

Building

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