

From:
Morris, Nevitt

To:
Xu, Lei (CBER); Zhu, Yao-Yao; Haudenschild, Changting

Cc:
Morris, Nevitt

Subject:
FW: BLA 125610, Clinical Registry Study Comment 11.27.17

Date:
Wednesday, December 06, 2017 3:57:14 PM

Attachments:
1.11.3 Response to Clinical Registry Study Comment received 27 Nov 17 -
RPE65 BLA.docx
image001.png
image004.png

(File Attachment comment)
(File Attachment comment)
(File Attachment comment)
Hello:

Please
see
the
email
below
and
attached
document
from
Spark
regarding
the
Clinical
Registry
Study
comment
submitted
to
them
on
November
27,
2017.
Thanks
Nevitt

From: Jim Wang
[mailto:jim.wang@sparktx.com]
Sent: Wednesday, December 06, 2017 3:49 PM
To: Morris, Nevitt
<Nevitt.Morris@fda.hhs.gov>
Subject: RE: BLA 125610, Clinical Registry Study Comment 11.27.17

Hi Nevitt,

Please see the attached Sponsor response to FDA's comment to collect long-term data on efficacy in the planned registry study. Spark does not agree with the recommendation to

include
efficacy
endpoints
in
the
proposed
registry
study
and
provided
rationale
in
the
attached
response
document.

Best
Regards,

Jim

Jim Wang, PhD, MBA

Head
of
Regulatory
Strategy
Spark
Therapeutics
Mobile:
609-613-0667
Office:
215-220-9293

www.sparktx.com

From: Morris,
Nevitt
[mailto:Nevitt.Morris@fda.hhs.gov]
(Unsigned signature field (Click to sign)) Signature field is unsigned

Sent: Monday,
November
27,
2017
11:26
PM
To: Jim
Wang
<jim.wang@sparktx.com>
Cc: Morris,
Nevitt
<Nevitt.Morris@fda.hhs.gov>
Subject: BLA
125610,
Clinical
Registry
Study
Comment
11.27.17

Hi Jim:

We recommend that you collect long-term data on efficacy, in the planned registry study. Additional assessment may include annual MLMT, VA, VF in addition to a full set of ophthalmological exam. Please submit a revised registry protocol for review when you are ready.

Please acknowledge receipt of this email.

Thanks

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
71,
Room
4207
10903
New
Hampshire
Avenue
Silver
Spring,
MD 20993
Phone: (240)
402-8269
Fax: (301)
595-1303
Nevitt.Morris@fda.hhs.gov

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