

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](http://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  
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