

From: Morris, Nevitt  
Sent: Tuesday, November 07, 2017 4:28 PM  
To: jim.wang@sparktx.com  
Cc: Morris, Nevitt  
Subject: BLA 125610 Spark Therapeutics, Information Request  
PVG/Clinical  
11.07.17

Importance: High

Hi Jim:

We have the following Clinical/Pharmacovigilance Information Request with a response requested by Tuesday November 14, 2017.

1. For the postmarket patient safety registry, please consider requiring at least 40 patients be enrolled and at least a 5-year period of enrollment. Having the study continue enrollment until both criteria are met would ensure the study includes a minimum number of patients. Also, consider requiring that the patients in the registry be examined by an ophthalmologist at least once per year.

2. In the Risk Management Plan, the patient safety registry is listed as a proposed additional pharmacovigilance activity for the category of missing information entitled "long term safety information (>8 years)" (pg. 40). Since the registry is following patients for 5 years, it will not be providing information on long term safety for >8 years. Please remove the patient safety registry from the list of proposed pharmacovigilance activities to address long term safety information (>8 years) or clarify in the table that the patient safety registry will be providing additional long term safety information for up to 5 years.

Thanks Jim and please acknowledge receipt of this email Information Request.

Nevitt

Nevitt Morris

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