

From: [Jarvis, Candace](#)
To: [James L'Italien, PhD \(jlitalien@avexis.com\)](#)
Cc: [Nancy Boman](#); [Xu, Lei \(CBER\)](#); [Singer, Mike](#); [Byrnes, Andrew](#); [Jarvis, Candace](#)
Subject: BLA 125694/0 | AveXis, Inc | Potential Post Marketing Requirement (PMR) |(Please Respond by May 13, 2019)
Date: Tuesday, May 07, 2019 9:50:49 AM
Attachments: [image013.png](#) **Importance:** High

Good morning Dr L'Italien,

In review of your current BLA 129694/0, we have the following Potential Post Marketing Requirement (PMR). Please respond to this email by email to me on May 13th as well as an amendment to the BLA.

Based on the uncertainty of what dose was administered in the Phase 1 trial (CL-101), we are considering a requirement for a post-marketing study. The study should be designed to assess both safety and efficacy with a sufficient follow-up duration and which evaluates 2 or more dose levels of onasemnogene abeparvovec-xioi in subjects with infantile-onset spinal muscular atrophy (SMA) with confirmed biallelic mutations in the *survival of motor neuron 1 (SMN1)* gene. Efficacy assessments should include survival; achievement of major developmental motor milestones such as independent sitting for at least 30 seconds, standing and walking; and ventilator use. Safety parameters should include hepatic abnormalities, platelet counts, and cardiac abnormalities, among others.

Please send us a proposed protocol synopsis to outline the post-market study by 10 am May 13. In the interim, we may have t-con to discuss this if you would like.

Please acknowledge receipt.

Regards,

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