

**From:** [Jarvis, Candace](#)  
**To:** [James L'Italien, PhD \(jlitalien@avexis.com\)](#)  
**Cc:** [Nancy Boman](#); [Jarvis, Candace](#); [Byrnes, Andrew](#); [Thompson, Deborah](#)  
**Subject:** BLA 125694/0 | AveXis, Inc. | Information Request 10 ( PLEASE Respond by November 30, 2018)  
**Date:** Friday, November 09, 2018 2:57:27 PM  
**Attachments:** [image002.png](#) **Importance:** High

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Good afternoon Dr L'Italien,

We have the following request for information regarding BLA 125694/0.

1. In the Pharmacovigilance Plan (PVP) (Routine Pharmacovigilance Activities, page 7), the sponsor indicates *AveXis will perform medical reviews to identify important single cases and conduct appropriate follow-up to obtain relevant medical information pertaining to these cases.* Please describe how cases will be identified as “important” and what will constitute “appropriate follow-up” and “relevant medical information.”
2. In the Pharmacovigilance Plan (PVP) (Routine Pharmacovigilance Activities, page 7), the sponsor indicates that guided questionnaires will be used for collecting data about specific safety concerns and that guided questionnaires will be developed for elevated transaminases, transient thrombocytopenia, and cardiac adverse events. Please submit copies of the guided questionnaires.
3. In the Protocol for AVXS-101-LT-001, the sponsor indicates under Section 7.4 Criteria for Study Termination that the study may be terminated by the sponsor. Please provide specific criteria/circumstances for the sponsor to terminate the study.
4. In the Protocol for AVXS-101-LT-001, Section 16 Data Handling and Recordkeeping indicates that electronic case report forms will be used. Copies of the electronic case report forms are not included in the protocol. Please submit copies of the electronic case report forms that will be used during years one to five of the study.
5. In the Protocol for AVXS-101-LT-001, Section 16 Data Handling and Recordkeeping does not indicate how missing data will be handled. Please explain how missing data will be handled.

Please respond to this request by November 30, 2018

Please confirm receipt.

Regards,

**Candace N. Jarvis**  
Regulatory Project Manager

*Center for Biologics Evaluation and Research*  
**Office of Tissues and Advanced Therapies**  
**U.S. Food and Drug Administration**

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