

**From:** [Jarvis, Candace](#)  
**To:** [James L'Italien](#)  
**Cc:** [Jarvis, Candace](#); [Byrnes, Andrew](#); [Singer, Mike](#); [Xu, Lei \(CBER\)](#)  
**Subject:** BLA 125694/0| AveXis, Inc| Information Request 8 (Please respond by November 7, 2018) **Date:** Friday, October 26, 2018 1:47:45 PM

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Good afternoon Dr. L'Italien,

We have the following request for information regarding BLA 125694. Please respond by November 7, 2018.

1. Please clarify any plans for screening patients for anti-AAV9 antibodies prior to the product administration during the post-licensure period.
  - a. What will AveXis's role be in screening patients for anti-AAV9 antibodies?
  - b. Will AveXis require that patients be screened for anti-AAV9 antibodies before shipping the product?
  - c. Will the assay described in SOP-283 be used?
  - d. Where will the assay be performed, and will the testing facility be CLIA certified?
2. Please clarify which assay for anti-AAV9 antibodies was used to screen subjects who were enrolled in study AVXS-101-CL-101
  - a. If subjects in AVXS-101-CL-101 were screened with a different assay, please clarify whether this assay and SOP-283 produce equivalent results.
3. Please clarify how the dose for each subject in Ph3 is determined.
4. Please provide your justification and supportive data for how to calculate dose for each patient, which is proposed on your PI (table 4).

Please confirm receipt of the email.

*Regards,*

**Candace N. Jarvis**

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*Center for Biologics Evaluation and Research*

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