

**From:** [Jarvis, Candace](#)  
**To:** [James L'Italien, PhD \(jlitalien@avexis.com\)](#)  
**Cc:** [Nancy Boman](#); [Byrnes, Andrew](#); [Jarvis, Candace](#)  
**Subject:** BLA 125694/0 \ AveXis, Inc. | Information Request 30 (Please Respond by January 28, 2019)  
**Date:** Friday, January 18, 2019 1:04:37 PM  
**Attachments:** [image002.png](#) **Importance:** High

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Good afternoon Dr. L'Italien,

We have the following CMC information request regarding BLA 125694/0. Please respond before or on January 28, 2019. Please let us know if you are unable to meet the deadline so that we can negotiate a new timeline for this request.

1. Regarding the vector reference standards described in 3.2.P.6:
  - a. When lot (b) (4) was being used as an interim reference standard, what was the SOP-137 (b) (4) of this standard? Table 1 in 3.2.P.6 only lists the (b) (4) measured with the NCH assay.
  - b. On what date was lot (b) (4) replaced as a standard by primary reference standard lot (b) (4)?
  - c. Please provide the full test report for the in vitro potency of lot (b) (4).
  - d. When lot (b) (4) is used as the reference standard in the in vitro potency assay SOP-347, is the potency of lot (b) (4) assigned to be 100%?
  - e. For the in vitro potency data reported in the stability sections of the BLA (3.2.S.7, 3.2.P.8 and RPT-411), which vector lot was used as the reference standard when generating these in vitro potency data?
2. Please provide the following documents:
  - a. NCR-441
  - b. CCR-028
  - c. REC-1070
  - d. RPT-840
  - e. The attachments that are mentioned in section 7 of RA-015
3. At the time that the BLA was submitted, a few test results were not available for inclusion in 3.2.P.5.6. If these data are now available, please update 3.2.P.5.6 to include the following:
  - a. In vitro potency results for lots (b) (4)
  - b. In vivo functionality results for lot (b) (4)

Please acknowledge receipt.

*Regards,*

***Candace N. Jarvis***

*Regulatory Project Manager*

*Center for Biologics Evaluation and Research*

**Office of Tissues and Advanced Therapies**

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