

From: [Jarvis, Candace](#)
To: [James L'Italien, PhD \(jlitalien@avexis.com\)](#)
Cc: [Nancy Boman](#); [Byrnes, Andrew](#); [Whatley, Angela](#); [Jarvis, Candace](#)
Subject: BLA 125694/0 |AveXis, Inc| Information Request 61 (PLEASE RESPOND BY MAY 7, 2019)
Date: Wednesday, May 01, 2019 10:40:17 PM **Attachments:**
[image013.png](#)

Good evening Dr L'Italien,

We have the following request for information regarding BLA 125694. Please respond no later than COB on May 7, 2019.

1. We note in section 3.2.p.7.1.3, you state that for each component of the container closure system (vial, stopper, and seal) quality control testing is performed by the supplier. And that prior to using in the manufacture AVXS-101 DP, AveXis quality control will conduct incoming testing and confirm the test results from the supplier. However, this section does not adequately specify which quality tests are performed by AveXis as part of incoming testing. Please clarify the confirmatory testing conducted by AveXis on the vial, stopper and seal which are used in the container closure system for the DP.
2. We note in section 3.2.p.7 you reference the secondary container with (b) (4) vials. Please update this section and others to reflect the current maximum number of vials which will be packaged in your secondary container.
3. The data provided do not support the normal operating range of (b) (4) because this study was conducted using (b) (4) which are not an adequate representations of the (b) (4) therefore the study does not support the operating range you proposed. The (b) (4) should be (b) (4) for a minimum of (b) (4) or you should provide data demonstrating that an alternative (b) (4) time for the (b) (4) that is reflective of your (b) (4) procedure results in adequate (b) (4).
4. The data provided do not support the normal operating range of (b) (4) for the filtered drug product (b) (4). Based on the data provided a homogenous solution with no significant differences in (b) (4) is not seen until at least (b) (4). Therefore, your normal operating range for the number of (b) (4) for the filtered drug product should include a minimum of (b) (4), preferably (b) (4).

Please update the information above and all previously agreed upon documents (SOPs, MBRs, operating ranges, process parameters, etc), in the BLA by May 7. Please also provide dates when the updated procedures will be implemented. 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
Tel: 240-402-8315
candace.jarvis@fda.hhs.gov



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