

From: [Jarvis, Candace](#)
To: [James L'Italien, PhD \(jlitalien@avexis.com\)](#)
Cc: [Nancy Boman](#); [Byrnes, Andrew](#); [Wang, Wei \(FDA\)](#); [Trout, Deborah](#); [Jarvis, Candace](#)
Subject: BLA 125694/0| AveXis, Inc| Information Request 39 (PLEASE RESPOND BY 3/22/19)
Date: Friday, March 08, 2019 3:01:27 PM
Attachments: [image002.png](#) **Importance:** High

Good afternoon Dr. L'Italien,

We have the following information regarding BLA 125694/0. Respond by March 22, 2019.

1. During the Pre-license Inspection (PLI) of your (b) (4) manufacturing facility (FEI: (b) (4)), you informed the FDA inspectors that the (b) (4) (FEI: (b) (4)) had not been equipped with refrigerators or freezers for the storage of (b) (4), and it was not currently being used for storage of materials or product. Please update the facility table in your application with the accurate usage information of the (b) (4) (FEI: (b) (4)).
2. You recently proposed to revise the patients' weight range from (b) (4) - 8.5) kg to (b) (4) - 13.5) kg. Please provide information on secondary packaging configurations and a shipping validation summary report for Drug Product dosed for patients' weight range between 8.5 kg - 13.5 kg.
3. Please provide a summary report of label validation studies to demonstrate that labels applied to frozen vials remain adhere and readable after thaw.
4. During the Pre-license Inspection (PLI) of your (b) (4) manufacturing facility (FEI: (b) (4)), you informed the FDA inspectors that, due to air pressure and air flow issues, you (b) (4)
(b) (4)
(b) (4) Please confirm.
5. We noted the following information discrepancy in your BLA submission (STN 125694/0) regarding the impurities in the (b) (4) PPQ lots (b) (4). Please provide a brief explanation of why the same set of data were reported differently in two sections of BLA submission.
In Table 3 of Section 3.2.P.5.4 of STN 125694/0 (page 9/13) it was shown that the Named Impurity (b) (4) was detected only in the (b) (4) PPQ lots (b) (4).
In Table 7 of Section 3.2.P.3.5 of STN 125694/0 (page 13/27) it was shown that the Named Impurity (b) (4) was detected in the (b) (4) PPQ

Lot (b) (4) and the Named Impurity (b) (4) was detected in the (b) (4)
PPQ Lot (b) (4)

Please confirm receipt.

Regards,

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