

From: [Jarvis, Candace](#)
To: [James L'Italien, PhD \(jlitalien@avexis.com\)](#)
Cc: [Nancy Boman](#); [Byrnes, Andrew](#); [Jarvis, Candace](#); [Thompson, Deborah](#)
Subject: BLA 125694/0| AveXis, Inc| Information Request 34 (PLEASE RESPOND BY February 28, 2019)
Date: Wednesday, February 13, 2019 10:56:32 AM
Attachments: [image002.png](#) **Importance:** High

Good morning Dr. L'Italien,

We have the following request for information regarding the 120 day safety update as submitted to BLA 125694. Please respond by February 28, 2019.

1. For Study AVXS-101-CL-304, CL-304 Listing 16.2.7.2 (Treatment-related AEs) indicates that Subject (b) (6) had an AE of elevated CPK that began on Study Day 14 and had an outcome of "not resolved." However, CL-304 Listing 16.2.8.1.2 (Laboratory - Chemistry) indicates that Subject (b) (6) had a creatine kinase level of 218 on Day 35 and a creatine kinase level of 150 on Study Day 60, which are both within normal limits (0-235 U/L). Please confirm the outcome of the AE "elevated CPK" for Subject (b) (6) in Study CL-304.
2. For Study AVXS-101-CL-304, Subject (b) (6) is listed as having an AE of elevated liver transaminases on Day 7 (Listing 16.2.7.1 of the Study AVXS-101304-TLFs- 120Day). Listing 16.2.8.1.2 (Laboratory- Chemistry) does not include lab values for liver transaminases on Day 7 or beyond. For Study CL-304, please provide liver transaminase lab values for Subject (b) (6) for Day 7 and beyond (if available).

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



THIS MESSAGE IS INTENDED ONLY FOR THE USE OF THE PARTY TO WHOM IT IS

ADDRESSED AND MAY CONTAIN INFORMATION THAT IS PRIVILEGED, CONFIDENTIAL, AND PROTECTED FROM DISCLOSURE UNDER LAW. If you are not the addressee, or a person authorized to deliver the document to the addressee, you are hereby notified that any review, disclosure, dissemination, copying, or other action based on the content of this communication is not authorized. If you have received this document in error, please immediately notify the sender immediately by e-mail or phone.