

From: [Jarvis, Candace](#)
To: [James L'Italien, PhD \(jlitalien@avexis.com\)](#)
Cc: [Nancy Boman](#); [Jarvis, Candace](#); [Byrnes, Andrew](#); [Galivo, Feorillo](#)
Subject: BLA 125694/0 | AveXis, Inc. | Information Request 25 (PLEASE RESPOND BY January 23, 2019)
Date: Wednesday, January 09, 2019 8:32:11 PM **Attachments:**
[image013.png](#)

Good evening Dr. L'Italien

We have the following request for information regarding the
Pharmacology/Toxicology review for BLA 125694

Developmental and Reproductive Toxicity (DART)

You state that DART testing was not relevant and therefore not conducted for AVXS101 (Sections 1.14.1.2 and 2.6.6). However, the AVXS-101 vector was detected and persisted in the gonads of administered mice for at least 24 weeks postadministration (Section 4.2.2.3 AD49TV.7A32.BTL). Also, human SMN1 transgene was expressed in the gonads of administered non-human primates at 6 months postadministration (Section 4.2.3.1. Nonhuman Primate - Intravenous). Please provide a scientific justification, with accompanying data, for not conducting DART testing for AVXS-101 for this BLA submission.

Please provide your response via email by COB Wednesday, January 23, 2018. The information should also be submitted as an amendment to the BLA.

Regards,

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