

From: [Jarvis, Candace](#)
To: [James L'Italien, PhD \(jlitalien@avexis.com\)](#)
Cc: [Nancy Boman](#); [Singer, Mike](#); [Xu, Lei \(CBER\)](#); [Thompson, Deborah](#); [Byrnes, Andrew](#); [Jarvis, Candace](#)
Subject: BLA 125694/0 | AveXis, Inc | Information Request 59 (PLEASE RESPOND BY MAY 6, 2019)
Date: Wednesday, May 01, 2019 10:02:18 PM
Attachments: [image013.png](#) **Importance:** High

Good evening Dr L'Italien

We have the following information request for BLA 125694/0. Please respond no later than COB on May 6, 2019.

1. *Technical Report RPT-1342 A Human Case Study: Biodistribution of AVXS-101 DNA and SMN Protein in Subject (b) (6) does not include analysis of brain tissue. Please explain why that information was not included.*
2. *For Study AVXS-101-CL-101, please provide a table (similar to Table 2 in the CL303 Efficacy and Safety Update) for all subjects in Cohort 2 indicating:*
 - a. *The visit month/age in months at which the subject reached the motor milestones of head control, sitting without support for at least 30 seconds, and walking.*
 - b. *The visit month/age in months for when the two (of seven) subjects who at baseline did not require enteral feeding, were first noted to require enteral feeding.*
 - c. *The visit month/age in months for when the four (of five) subjects who at baseline required enteral feeding, were first noted to be able to feed orally.*
 - d. *For the eleven subjects who were able to feed orally by the end of the study, the visit month/age in months for when the six of those subjects were able to exclusively feed orally.*

If the specific date is unknown, please indicate so, and provide the date at which the milestone or event was first recorded for the study.

Please confirm receipt.

Regards,

Candace N. Jarvis
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