

From: [Jarvis, Candace](#)
To: [James L'Italien, PhD \(jlitalien@avexis.com\)](#)
Cc: [Jarvis, Candace](#); [Byrnes, Andrew](#); [Singer, Mike](#); [Xu, Lei \(CBER\)](#)
Subject: BLA 125694/0| AveXis, Inc| Information Request 6 (Please RESPOND BY November 2, 2018)
Date: Friday, October 19, 2018 7:34:00 PM
Attachments: [image002.png](#) **Importance:** High

Good evening Dr. L'Italien,

We have the following request for information regarding BLA 125694:

1. For all studies for which data is being submitted, please clarify in detail: the protocol for how study investigators determined the hours of ventilatory support used by the study subjects; which study investigators made these determinations; how often these determinations were made; and how many days each determination covered. Please also clarify whether the subjects' ventilatory support use was guided by study investigators, or by physicians not involved in the study.
2. Please provide a single table with data for all subjects in Study AVXS-101-CL-101, describing for each study time point, the hours of ventilatory support used (night, day, and total). Please also clarify the abbreviation "NS" (e.g., used in Listing 16.2.6.2.2-20).
3. Please specify the number of subjects (and duration of follow-up) from the Phase 3 trial for whom efficacy data will be available in the 4-month safety and efficacy supplement.
4. Please help us identify the following subject-level data for Study 303 in the submission: a. Data on daily ventilator use
b. Milestone achievement at cutoff
5. Please provide us an update on the number of subjects enrolled and treated. Please include the date of treatment and the study site of each subject.

Please respond to this information request by November 2, 2018.

Please acknowledge receipt of this email.

Regards,

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