

**From:** [Jarvis, Candace](#)  
**To:** [James L'Italien, PhD \(jlitalien@avexis.com\)](#)  
**Cc:** [Jarvis, Candace](#); [Byrnes, Andrew](#)  
**Subject:** BLA 125694/0| AveXis, Inc Information Request 1 (Please Respond by 10/9/18)  
**Date:** Friday, October 05, 2018 10:47:50 AM  
**Attachments:** [image002.png](#) **Importance:** High

---

Good morning Dr. L'Italien,

While conducting a preliminary review of your recent BLA submitted under 125694, we noted that product name cited on your 356h form (onasemnogene abeparvovec) is different than the product name cited in the September 30, 2014 Orphan Drug Designation letter (adeno-associated virus serotype 9 expressing the human Survival Motor Neuron gene). Please amend as soon as possible the cover letter and the 356h form to assert that the drug in BLA 125694 is indeed "adeno-associated virus serotype 9 expressing the human Survival Motor Neuron gene" only for the indication of treating spinal muscular atrophy, as designated by FDA Orphan Product Office.

Please let me know if you have any questions.

*Regards,*

**Candace N. Jarvis**  
*Sr. 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](mailto: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.