

**MEMORANDUM**  
**Department of Health and Human Services**  
**Public Health Service**  
**Food and Drug Administration**  
**Center for Biologics Evaluation and Research**

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**Date:** March 25, 2019

**From:** Sonny Saini, Pharm.D., MBA  
Regulatory Review Officer  
Advertising and Promotional Labeling Branch (APLB)  
Division of Case Management

**Through:** Lisa L. Stockbridge, Ph.D.  
Branch Chief  
Advertising and Promotional Labeling Branch (APLB)  
Division of Case Management

**To:** Candace Jarvis, Regulatory Project Manager  
Andrew Byrnes, Ph.D., Chair

**Subject:** Labeling Review  
**ZOLGENSMA (onasemnogene abeparvovec-xioi)**  
**BLA: 125694/0**  
Sponsor: AveXis, Inc.

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**Background:** The sponsor submitted:

<input type="checkbox"/>	New Approval
<input type="checkbox"/>	Changes Being Effectuated (CBE) supplement
<input checked="" type="checkbox"/>	Prior Approval Supplement (PAS) Amendment
<input type="checkbox"/>	Major Amendment

Submission contains:

<input checked="" type="checkbox"/>	Prescribing Information (PI)
<input type="checkbox"/>	Patient Package Insert (PPI)
<input checked="" type="checkbox"/>	Carton and/or container labels
<input type="checkbox"/>	Other

Submission Date: October 3, 2018

PDUFA action Date: May 31, 2019

### **APLB Comments/Recommendations**

This labeling review is for a Prior Approval Supplement (PAS) that AveXis, Inc. submitted on October 3, 2018 for ZOLGENSMA (onasemnogene abeparvovec-xioi) (STN 125694/0). In this original application, AveXis is seeking approval for ZOLGENSMA (onasemnogene abeparvovec-xioi) as an adeno associated virus (AAV) vector-based gene therapy indicated for the treatment of pediatric patients with infantile-onset spinal muscular atrophy (SMA) with confirmed biallelic mutations in the survival motor neuron 1 (SMN1) gene.

We also refer to draft labeling sent to APLB by OTAT on March 6, 2019 and revised carton/container labeling sent to APLB on February 26, 2019.

Upon reviewing this application, APLB has the following comments from a promotion and comprehension perspective.

### **GENERAL**

- Use active voice throughout label.
- Refrain from using statements regarding the practice of medicine.

### **HIGHLIGHTS**

- Insert year of initial U.S. approval in Highlights.
- Throughout the Highlights section of the label use the term suspension as dosage form for ZOLGENSMA instead of solution.
- Delete in Highlights subsection under DOSAGE and ADMINISTRATION titled Preparation and Administration of Intravenous Infusion. This subsection should remain in the Full Prescribing Information (FPI).

### **TABLE OF CONTENTS**

Ensure that the TABLE OF CONTENTS is consistent with the FPI.

### **FULL PRESCRIBING INFORMATION**

- Throughout the FPI use the term suspension as dosage form for ZOLGENSMA instead of solution.
- Under WARNINGS AND PRECAUTIONS – 5.1 Elevated Aminotransferases, edit ‘maybe serious’ to ‘may be serious’.

### **CARTON/CONTAINER LABELING**

APLB reviewed the draft carton/container labeling for ZOLGENSMA from a promotional and comprehension perspective and does not have any proposed changes.

The above comments have been provided as a labeling consult. If you have any questions regarding this review please contact Sonny Saini, Regulatory Review Officer, at [sonny.saini@fda.hhs.gov](mailto:sonny.saini@fda.hhs.gov).