



**U.S. FOOD & DRUG**  
ADMINISTRATION

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## Memorandum

Date: October 22, 2018

From: Oluchi Elekwachi, PharmD, MPH, Regulatory Review Officer  
OCBQ/DCM/APLB

Through: Lisa Stockbridge, PhD, Branch Chief  
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To: Candace Jarvis, RPM, OMPT/CBER/OTAT/DRPM/RPMBII  
Mike Singer, Medical Officer, OMPT/CBER/OTAT/DCEPT/GMBI

Subject: Review of Proposed Proprietary Name “**ZOLGENSMA**” (onasemnogene  
abeparvovec) solution for intravenous infusion

BLA 125694  
Applicant: AveXis, Inc

Recommendation: **ZOLGENSMA – Acceptable**

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## Executive Summary

The Advertising and Promotional Labeling Branch (APLB) has completed the review of the proposed proprietary name, **ZOLGENSMA**, a gene replacement therapy for Spinal Muscular Atrophy (SMA). We recommend that the proposed proprietary name, **ZOLGENSMA**, be found **Acceptable**.

According to SOPP 8001.4 Review of CBER Regulated Product Proprietary Names, the product office, Office of Tissues and Advanced Therapies (OTAT), makes the final decision on the acceptability of a proposed proprietary name. To meet the PDUFA performance goal, OTAT must communicate this decision to the applicant within 90 days of the receipt of the proprietary name review (PNR) submission. The PDUFA goal date for this PNR is December 29, 2018.

If OTAT accepts our recommendation that the proposed primary proprietary name, **ZOLGENSMA**, be found Acceptable, we offer the following communication-ready language:

*In consultation with CBER's Advertising and Promotional Labeling Branch, we conclude that under the Federal Food, Drug, and Cosmetic Act and applicable regulations, your proposed*

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*proprietary name, **ZOLGENSMA**, is Acceptable.*

OTAT is responsible for communicating CBER's decision to the Applicant and should enter the communication issuance date into RMS-BLA before December 29, 2018, in order to meet the deadline and stop the performance clock. Please notify APLB when this action has been completed.

## **Background**

On October 1, 2018, AveXis, Inc. (AveXis) submitted the proposed proprietary name, **ZOLGENSMA**, for its an adeno-associated virus (AAV) vector-based gene replacement therapy. According to the applicant, the name **ZOLGENSMA** (pronounced zole jen' smah) was derived from the intended use, **gene** replacement therapy for **SMA**. The indication is for the treatment of pediatric patients with spinal muscular atrophy (SMA) Type 1 with or without disease onset.

**ZOLGENSMA** will be provided as a  $1.1 \times 10^{14}$  vg/kg solution for one-time-only intravenous infusion in pediatric patients with a body weight of up to 8 kg. **ZOLGENSMA** should be administered as a slow infusion over 30 - 60 minutes, not an intravenous push or bolus.

**ZOLGENSMA** will be dispensed from specialty pharmacies or directly by the patient physician. For use within 14 days, the product will be delivered frozen, stored at 2° to 8°C (35° to 46°F) and protected from light.

## **Method**

APLB utilized the FDA Phonetic and Orthographic Computer Analysis (POCA) and the following databases:

1. CBER list of Licensed Products ending October 11, 2018, at <http://www.fda.gov/downloads/BiologicsBloodVaccines/UCM149970.pdf>
2. DailyMed at <http://dailymed.nlm.nih.gov/dailymed/about.cfm>
3. Drugs@FDA current through October 11, 2018, at <http://www.accessdata.fda.gov/scripts/cder/drugsatfda>
4. Electronic Orange Book current through October 11, 2018, at <http://www.accessdata.fda.gov/scripts/cder/ob/default.cfm>
5. Google Internet search at <http://www.google.com>
6. Micromedex at <http://www.micromedexsolutions.com/micromedex2/librarian>

7. United States Patent and Trademark Office (USPTO) at <http://www.uspto.gov/trademarks/index.jsp>
8. USAN Stem at <http://www.ama-assn.org/ama1/pub/upload/mm/365/stem-list-cumulative.pdf>

APLB also consulted the review team on the proprietary name and incorporated their recommendations into this review.

## **Results**

### **1. Prescreening for Objectionable Naming Practices**

The proposed proprietary name, **ZOLGENSMA**, was screened against the following:

- Obvious similarities in spelling and pronunciation
- Manufacturing characteristics
- Medical and/or coined abbreviations
- Inert or inactive ingredients
- Combination of active ingredients
- United States Adopted Name (USAN) stems
- Same proprietary name for products containing different active ingredients
- Reuse of proprietary names
- Dosage form or route of administration
- Dosing interval
- Established or proper name
- Modifiers as components of a proprietary name
  - Use of numerals as modifiers
  - Device-related modifiers
  - Descriptive modifiers
- Brand name extensions (Umbrella branding)
- Dual proprietary names
- Foreign drug proprietary name
- Prescription-to-OTC switch
- Use of symbols
- Incorporation of the Applicant's name

### **2. Evaluating for Promotional and Safety Concerns**

#### **a. Promotional Review [21 CFR 201.10 (c)(3), 202.1 (e)(5)(i), and (e)(6)(i)]**

The proposed proprietary name, **ZOLGENSMA**, is not regarded to be false, misleading or fanciful.

#### **b. Look-alike Sound-alike Safety Review [21 CFR 201.10 (c)(5)]**

Since drug products are prescribed through written, verbal, and/or electronic orders, such forms of communication may lead to medication errors, particularly if proprietary or established names sound or

look alike. APLB conducted a search using POCA, with DPRF, Drugs@FDA, Cerner US Legend and OTC, CBER Biologic, Orange Book, and RxNorm as data sources, to identify existing names of concern with potential combined orthographic and phonetic similarity to **ZOLGENSMA** and found 223 other moderately similar names. However, different dosage form and strength mitigate the concern over confusion of these products with **ZOLGENSMA**.

Thus, APLB recommends that **ZOLGENSMA** be found **Acceptable**.

If you have any questions regarding this review please contact Oluchi Elekwachi, PharmD, MPH Regulatory Review Officer, at 240-402-8930.