



U.S. FOOD & DRUG
ADMINISTRATION

Memorandum

Date: January 23, 2019

From: Lisa L. Stockbridge, Ph.D.
Branch Chief
OCBQ/DCM/APLB

To: Candace Jarvis, RPM, OMPT/CBER/OTAT/DRPM
Tejashri Purohit-Sheth, Director OMPT/CBER/OTAT/DCEPT

Subject: PROPER NAME SUFFIX RECOMMENDATION
STN 125694/0
Sponsor: Avexis Inc.

Background

On November 28, 2018, Avexis Inc. (Avexis) submitted proposed proper name suffixes to their BLA 125694, ZOLGENSMA (onasemnogene abeparvovec - xxxx), a gene therapy product for the treatment of Spinal Muscular Atrophy (SMA). An analysis conducted by the Drug Safety Institute was included in the submission.

ZOLGENSMA (onasemnogene abeparvovec – xxxx) is an adeno-associated virus expressing a human Survival Motor Neuron gene. The proposed proper name suffixes for review, listed in Avexis' order of preference are: -(b) (4) -xioi.

Assessment of the proper name with suffix

The proposed suffixes were evaluated using the criteria set forth in *Guidance for Industry – Nonproprietary Naming of Biological Products*. A suffix should be unique, devoid of meaning, composed of four lowercase letters of which at least three are distinct, nonproprietary, and free of legal barriers that would restrict its usage. A suffix should not include numbers or symbols, be false or misleading with respect to safety or efficacy of the product, include abbreviations commonly used in clinical practice in a manner that may lead it to be misinterpreted as another element on the prescription or order, contain or suggest a drug substance name or core name, look similar to or have the potential to be mistaken for the name of a currently marketed product, connote the name of the license holder, or be too similar to another FDA-designated suffix.

Using the above criteria, Avexis' first five proposed proper name suffixes are not devoid of
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meaning for the following reasons:

- Directly referencing the applicant's name, Avexis (e.g., - (b) (4))
- Connoting the product's mechanism of action and indication, gene therapy or gene replacement therapy (e.g., (b) (4))

Recommendation

Avexis' proposed proper name suffixes were reviewed, and the first five choices were unacceptable. Thus, we offer the following letter-ready language to convey to the applicant:

Your first five proposed suffixes are unacceptable in one or both of the following ways:

- *Suffix connotes your name, Avexis (e.g., - (b) (4))*
- *Suffix connotes your product's mechanism of action and/or indication, gene therapy or gene replacement therapy (e.g., (b) (4))*

Your sixth proposed suffix meets the criteria set forth in Guidance for Industry – Nonproprietary Naming of Biological Products. Thus, we find the proper name, onasemnogene abeparvovec – xioi, conditionally acceptable for your proposed product. Should your 351(a) BLA be approved during this review cycle, onasemnogene abeparvovec – xioi, will be the proper name designated in the license and you should revise your proposed labels and labeling accordingly. However, please be advised that if your application receives a complete response, the acceptability of your proposed suffix will be re-evaluated when you respond to the deficiencies. If we find your proposed proper name unacceptable upon our re-evaluation at that time, we will inform you of our finding.

Firm name: Avexis

BLA: 125694

Letter type: Suffix Memorandum

History

Draft:	L. Stockbridge	01/18/19
Concur:	T. Purohit-Sheth	01/22/19
Concur:	M. Mendoza	01/23/19
Finalized:	L. Stockbridge	01/23/19

Concurrence box:

MailCode or Office	Name	Approval
APLB	L. Stockbridge	
OCBQ	M. Mendoza	