



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

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## Memorandum

Date: September 26, 2022

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OCBQ/DCM/APLB

To: Shalini Seetharaman, CBER/OTAT/DRPM/RPMB4  
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Megan Zimmerman, MD, CBER/OTAT/DCEPT/MHB

Subject: PROPER NAME SUFFIX RECOMMENDATION  
STN 125772/0  
Sponsor: CSL Behring, LLC

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## **Background**

On March 21, 2022, CSL Behring, LLC (CSL) submitted proposed proper name suffixes for BLA125772, HEMGENIX (etranacogene dezaparvovec). HEMGENIX is a gene therapy product consisting of a recombinant adeno-associated viral vector, serotype 5, containing the Padua variant of human coagulation Factor IX (AAV5-hFIX). It will be provided as a solution for intravenous infusion, indicated to reduce the frequency of bleeding episodes (b) (4) in adults with hemophilia B (congenital Factor IX deficiency) (b) (4).

The proposed proper name suffixes for review, listed in order of preference, are: *-ixgt, -hbgt, -gthb, -fixb, -gtbr, -gtbe, -hemb, -hmbi, -frdm, -bfix*

Please note that this is the second suffix review memorandum for BLA 125772. A July 5, 2022, review of the proposed suffixes failed to appropriately adjudicate the suffix – *frdm* as unacceptable. The review committee considers this suffix unacceptable for the reasons cited below.

## **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, CSL's the following proposed proper suffixes are not devoid of meaning and are unacceptable for the following reasons:

- Connoting the class of product, **gene therapy**: **-ixgt**, **-hbgt**, **-gthb**, **-gtbr**, **-gtbe**
- Connoting the indication of the product, **hemophilia B**, **Factor IX**: **-ixgt**, **-hbgt**, **-gthb**, **-fixb**, **-hemb**, **-hmbi**
- The ninth suffix, **-frdm**, is unacceptable because it looks and sounds like the word *freedom*, which overstates the efficacy of this product and is not devoid of meaning. Similarly, the fourth and tenth proposed suffixes, **-fixb** and **-bfix**, also are unacceptable because they connote the indication in a way that misrepresents the efficacy of the product (i.e., HEMGENIX does not cure hemophilia B).

## **Recommendation**

CSL's proposed proper name suffixes were reviewed and all ten were found unacceptable. Thus, we offer the following letter-ready language to convey to the applicant:

*The Center for Biologics Evaluation and Research's Office of Tissues and Advanced Therapies and Office of Compliance and Biologics Quality have reviewed your ten proposed proper name suffixes, and we find all of them unacceptable.*

*The proposed suffixes were evaluated using the criteria set forth in Guidance for Industry Nonproprietary Naming of Biological Products (<https://www.fda.gov/downloads/drugs/guidances/ucm459987.pdf>). 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, your proposed suffixes are not devoid of meaning and thus were found unacceptable for the following reasons:*

- *Connotes the class of product, **gene therapy**: **-ixgt**, **-hbgt**, **-gthb**, **-gtbr**, **-gtbe**,*
  - *Connotes the indication of the product, **hemophilia B**, **Factor IX**: **-ixgt**, **-hbgt**, **-gthb**, **-fixb**, **-hemb**, **-hmbi***
  - *The suffix, **-frdm**, is unacceptable because it looks and sounds like the word *freedom*, which overstates the efficacy of this product and is not devoid of meaning. Similarly, the proposed suffixes,*
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- **fixb** and – **bfix**, are unacceptable because they connote the indication in a way that misrepresents the efficacy of the product (i.e., HEMGENIX does not cure hemophilia B).

*Because your proposed suffixes were found unacceptable, you may provide us with additional proposed suffixes for consideration. Please be informed that, in the absence of an acceptable alternative proposal, we intend to assign a randomly generated, pre-screened suffix for inclusion in the proper name if your product is licensed within this review cycle.*

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