

BLA 022458/S-029

## SUPPLEMENT APPROVAL

Pfizer Inc.  
Attention: Deneen Stewart  
Director, Pfizer Global Regulatory Affairs  
500 Arcola Road  
Collegetown, PA 19426

Dear Ms. Stewart:

Please refer to your supplemental biologics license application (sBLA), received March 18, 2022, and your amendments, submitted under section 351(a) of the Public Health Service Act for Elelyso (taliglucerase alfa) injection.

This Prior Approval supplemental biologic license application provides for a revision to the Dosage and Administration section of the Prescribing Information, Section 2.3 Administration Instructions, to update infusion rate for pediatric patients so that the endotoxin levels are maintained below the safety threshold for Elelyso.

### **APPROVAL & LABELING**

We have completed our review of this application, as amended. It is approved, effective on the date of this letter, for use as recommended in the enclosed agreed-upon labeling with minor editorial revisions listed below and reflected in the enclosed labeling.

- In the Highlights of the Prescribing Information, leading zeros were removed for the month of the revision dates in Recent Major Changes and the Revised date. The dates now appear as 8/2022.
- In Section 8.1 Pregnancy of the Prescribing Information, two inter-subsection 8.1 cross-references were revised to use parentheses instead of brackets as follows: (*see Clinical Considerations*) and (*see Data*).

### **CONTENT OF LABELING**

As soon as possible, but no later than 14 days from the date of this letter, submit, via the FDA automated drug registration and listing system (eLIST), the content of labeling [21 CFR 601.14(b)] in structured product labeling (SPL) format, as described at FDA.gov,<sup>1</sup> that is identical to the enclosed labeling (text for the Prescribing Information)

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<sup>1</sup> <http://www.fda.gov/ForIndustry/DataStandards/StructuredProductLabeling/default.htm>

and include the labeling changes proposed in any pending “Changes Being Effectuated” (CBE) supplements.

Information on submitting SPL files using eLIST may be found in the guidance for industry *SPL Standard for Content of Labeling Technical Qs and As*.<sup>2</sup>

The SPL will be accessible via publicly available labeling repositories.

Also within 14 days, amend all pending supplemental applications that include labeling changes for this BLA, including pending “Changes Being Effectuated” (CBE) supplements, for which FDA has not yet issued an action letter, with the content of labeling [21 CFR 601.12(f)] in Microsoft Word format that includes the changes approved in this supplemental application, as well as annual reportable changes. To facilitate review of your submission(s), provide a highlighted or marked-up copy that shows all changes, as well as a clean Microsoft Word version. The marked-up copy should provide appropriate annotations, including supplement number(s) and annual report date(s).

## **REQUIRED PEDIATRIC ASSESSMENTS**

Under the Pediatric Research Equity Act (PREA) (21 U.S.C. 355c), all applications for new active ingredients (which includes new salts and new fixed combinations), new indications, new dosage forms, new dosing regimens, or new routes of administration are required to contain an assessment of the safety and effectiveness of the product for the claimed indication(s) in pediatric patients unless this requirement is waived, deferred, or inapplicable.

Because this drug product for this indication has an orphan drug designation, you are exempt from this requirement.

## **REPORTING REQUIREMENTS**

We remind you that you must comply with reporting requirements for an approved BLA (in 21 CFR 600.80 and in 21 CFR 600.81).

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<sup>2</sup> We update guidances periodically. For the most recent version of a guidance, check the FDA Guidance Documents Database <https://www.fda.gov/RegulatoryInformation/Guidances/default.htm>.

If you have any questions, contact Jenny Doan, Regulatory Project Manager at (301) 796-1023.

Sincerely,

*{See appended electronic signature page}*

Yuliya Yasinskaya, MD  
Deputy Director for Safety (acting)  
Division of Rare Diseases and Medical Genetics  
Office of Rare Diseases, Pediatrics, Urologic and  
Reproductive Medicine  
Center for Drug Evaluation and Research

ENCLOSURE:

- Content of Labeling
  - Prescribing Information

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**This is a representation of an electronic record that was signed electronically. Following this are manifestations of any and all electronic signatures for this electronic record.**  
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/s/  
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YULIYA I YASINSKAYA  
08/30/2022 09:58:30 AM