

BLA 761204/S-004

## SUPPLEMENT APPROVAL

Amicus Therapeutics US, LLC  
Attention: Monique Fleck  
Associate Director, Global Regulatory Affairs  
3675 Market Street  
Philadelphia, PA 19104

Dear Monique Fleck:

Please refer to your supplemental biologics license application (sBLA), dated and received January 29, 2024, and your amendments, submitted under section 351(a) of the Public Health Service Act for Pombiliti (cipaglusosidase alfa-atga) injection.

This Prior Approval supplemental biologics application provides for adding hypotension and somnolence as new adverse reactions in Section 6.1 of the Prescribing Information, as well as providing for additional editorial updates.

### **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.

### **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) 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.

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

<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>.

Also within 14 days, amend all pending supplemental applications that include labeling changes for this BLA, including pending "Changes Being Effected" (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 none of these criteria apply to your application, 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).

If you have any questions, contact Jenny Doan, Regulatory Project Manager, at (301) 796-1023 or [Jenny.Doan@fda.hhs.gov](mailto:Jenny.Doan@fda.hhs.gov).

Sincerely,

*{See appended electronic signature page}*

Catherine Pilgrim-Grayson, MD, MPH  
Director (Acting)  
Division of Rare Diseases and Medical Genetics  
Office of Rare Diseases, Pediatrics, Urology 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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CATHERINE A PILGRIM-GRAYSON  
07/25/2024 08:31:00 AM