



BLA 125291/S-156
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SUPPLEMENT APPROVAL

Genzyme Corporation
Attention: Sudhakarr Balasubramanian
Manager, Regulatory Affairs
55 Corporate Drive, Mail Stop: 55C-300
Bridgewater, NJ 08807

Dear Mr. Balasubramanian:

Please refer to your supplemental biologics license applications (sBLAs), received November 12, 2021, and your amendments, submitted under section 351(a) of the Public Health Service Act for Lumizyme (alglucosidase alfa) injection.

These supplemental biologics license applications provide for the following changes to the Prescribing Information:

- S-156:** Updates to Clinical Pharmacology section 12.3 Pharmacokinetics based on the results of the postmarketing study AGLU07710/MSC12790 titled “Pharmacokinetics of Alglucosidase Alfa in Patients with Pompe Disease (PAPAYA).”
- S-157:** Safety-related labeling changes to Dosage and Administration section 2.2 Instruction for Use to add the infusion rate for infants weighing less than 1.88 kg.

APPROVAL & LABELING

We have completed our review of these applications, as amended. They are 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,¹ that is identical to the enclosed labeling (text for the Prescribing Information)

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

and include the labeling changes proposed in any pending “Changes Being Effected” (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*.²

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

FULFILLMENT OF POSTMARKETING COMMITMENT

We have received your submission dated May 19, 2021, containing the final report for the following postmarketing commitment listed in the May 24, 2010, approval letter for BLA 125291 for Lumizyme (alglucosidase alfa):

2330-6	A prospective pharmacokinetic (PK) study will be conducted to characterize the pharmacokinetics of Lumizyme (alglucosidase alfa) in pediatric patients in the age range of 8 years to 18 years.
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We have reviewed your submission and conclude that the above postmarketing commitment was fulfilled.

² 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>.

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We remind you that there are postmarketing requirements and postmarketing commitments listed in the May 24, 2010, approval letter that are still open.

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 CAPTAIN 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

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.

/s/

YULIYA I YASINSKAYA
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