BLA 761261/S-004

SUPPLEMENT APPROVAL

Genzyme Corporation
Attention: Swetha Kalidindi
Director, Regulatory Strategist
55 Corporate Drive,
Bridgewater, New Jersey 08807

Dear Swetha Kalidindi:

Please refer to your supplemental biologics license application (sBLA), dated and received September 27, 2024, and your amendments, submitted under section 351(a) of the Public Health Service Act for Xenpozyme (olipudase alfa-rpcp) injection.

This "Changes Being Effected" supplemental biologics application provides for updates to the Prescribing Information for Xenpozyme to include the following in Section 2 DOSAGE AND ADMINISTRATION:

- Sub-section 2.4 Missed Doses: Additional information in Table 3 regarding three or more consecutive missed doses.

- Sub-section 2.7 Administration Instructions: Additional instruction on dose and infusion rate for Xenpozyme under the heading Home Infusion.

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,[1] that is identical to the enclosed labeling (text for the Prescribing Information) and include the labeling changes proposed in any pending "Changes Being Effected" (CBE) supplements.

[1] http://www.fda.gov/ForIndustry/DataStandards/StructuredProductLabeling/default.htm

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.

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).

[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.

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

Sincerely,

{See appended electronic signature page}

Catherine Pilgrim-Grayson, MD, MPH
Director
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

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/

CATHERINE A PILGRIM-GRAYSON
12/20/2024 06:31:08 PM