BLA 125117/S-132

SUPPLEMENT APPROVAL

BioMarin Pharmaceutical Inc.
Attention: Robin Rolser
Senior Director, Regulatory Affairs Global Labeling
105 Digital Drive
Novato, CA 94949

Dear Robin Rolser:

Please refer to your supplemental biologics license application (sBLA), dated and received April 29, 2024, and your amendments, submitted under section 351(a) of the Public Health Service Act for Naglazyme (galsulfase) injection.

This Prior Approval supplemental biologics application provides for updates to the Prescribing Information as follows:

- Section 8, USE IN SPECIFIC POPULATIONS, is updated with information from the pregnancy sub-study conducted to fulfill postmarketing commitment (PMC) 2599-9.

- Relocation of immunogenicity information from Section 6, ADVERSE REACTIONS, to Section 12, CLINICAL PHARMACOLOGY, in accordance with FDA guidance.

APPROVAL & LABELING

We have completed our review of this application. 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.

- To adhere to the Selected Requirements of the Prescribing Information (SRPI), the period which followed the Prescribing Information's Table of Contents heading is deleted prior to the asterisk as follows: FULL PRESCRIBING INFORMATION: CONTENTS*

- In the Full Prescribing Information, the brackets enclosing the cross-reference in paragraph 2 of subsection 12.6, Immunogenicity, are italicized as follows: *[see Clinical Studies (14)]*

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.

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 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).

[1] http://www.fda.gov/ForIndustry/DataStandards/StructuredProductLabeling/default.htm
[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, contact Avinash Kalsi, Regulatory Project Manager, at (301) 348-1432 or avinash.kalsi@fda.hhs.gov.

Sincerely,

{See appended electronic signature page}

Yuliya Yasinskaya, MD
Deputy Director
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/

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