BLA 761079/S-010

SUPPLEMENT APPROVAL /
FULFILLMENT OF POSTMARKETING REQUIREMENT

BioMarin Pharmaceuticals Inc.
Attention: Paul Pisacane
Director, Regulatory Affairs
105 Digital Drive
Novato, CA 94949

Dear Mr. Pisacane:

Please refer to your supplemental biologics license application (sBLA), dated and received May 21, 2020, and your amendments received on October 19, 2020, submitted under section 351(a) of the Public Health Service Act for Palynziq (pegvaliase-pqpz) injection.

This Prior Approval supplemental biologics application provides for revisions to section 8.1 of the Prescribing Information (PI) to include information from the required postmarking non-clinical study BMN165-18-080, entitled "A Peri-/Post-natal Development Study of rAvPAL-PEG Administered by Subcutaneous Injection in Rats with Additional Developmental Assessments in F1 Generation."

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.

The Revised date of the HIGHLIGHTS OF PRESCRIBING INFORMATION was update to 11/2020.

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 none of these criteria apply to your supplemental application, you are exempt from this requirement.

FULFILLMENT OF POSTMARKETING REQUIREMENT

We have received your submission dated May 21, 2020, containing the final report for the following postmarketing requirement listed in the May 24, 2018, approval letter for BLA 761079.

3349-3 Pre-/Postnatal development study in rats treated with pegvaliase-pqpz using a set of testing methods that is sufficient to evaluate postnatal development, including the evaluation of physical developmental parameters and tests for effects on behavior, motor activity, sensory or sensorimotor functions, and reflex development.

[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.

We have reviewed your submission and conclude that the above requirement was fulfilled.

We remind you that there are postmarketing requirements and postmarketing commitments listed in the May 24, 2018, 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 Nicolas Kong, Regulatory Project Manager at Nicolas.Kong@fda.hhs.gov or 240-402-0269.

Sincerely,

{See appended electronic signature page}

Kathleen Donohue, M.D.
Division Director
Division of Rare Diseases and Medical Genetics (DRDMG)
Office of Rare Diseases, Pediatrics, Urologic and Reproductive Medicine (ORPURM)
Center for Drug Evaluation and Research

ENCLOSURES:

- Content of Labeling
  - Prescribing Information
  - Medication Guide (previously approved October 6, 2020)
  - Instructions for Use (previously approved October 6, 2020)
- REMS (previously approved October 6, 2020)

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/

KATHLEEN M DONOHUE
11/20/2020 01:58:04 PM