BLA 761052/S-007

**SUPPLEMENT APPROVAL**

BioMarin Pharmaceutical
Attention: Elizabeth A. Moyle
Executive Director, Regulatory Affairs Global Labeling
105 Digital Drive
Novato, CA 94949

Dear Ms. Moyle:

Please refer to your supplemental biologics license application (sBLA), dated June 28, 2019, submitted under section 351(a) of the Public Health Service Act for Brineura (cerliponase alfa) injection.

This "Changes Being Effected" supplemental biologics application provides for revisions to section 2.1 Important Preparation and Administration Information in the Prescribing Information to include the specific B. Braun Perfusor® Space Infusion Pump System cleared for use with Brineura and the essential performance requirements for a syringe pump used to deliver Brineura.

## 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.

- Update the date in Recent Major Changes and the revision date at the end of Highlights in the Prescribing Information to the approval date.

## 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 (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).

**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}*

Dragos G. Roman, MD
Acting Director
Division of Gastroenterology and Inborn Errors Products
Office of Drug Evaluation III
Center for Drug Evaluation and Research

ENCLOSURE(S):

- Content of Labeling
    - Prescribing Information

[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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/s/

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DRAGOS G ROMAN
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