NDA 203389/S-022

SUPPLEMENT APPROVAL

Horizon Pharma USA Inc.
Attention: Ingrid Hoos
Vice President, Regulatory Affairs
150 S Saunders Road
Lake Forest, IL 60045

Dear Ms. Hoos:

Please refer to your supplemental new drug application (sNDA) dated August 3, 2019, and received on August 6, 2019, and your amendments, submitted pursuant to section 505(b)(2) of the Federal Food, Drug, and Cosmetic Act (FDCA) for Procysbi® (cysteamine bitartrate) delayed release capsules.

This Prior Approval supplemental new drug application provides for the following updates to the prescribing information:

- safety and efficacy information in *Section 6 Adverse Reactions* and *Section 14 Clinical Studies*, respectively, based on final analysis of a long-term, open-label extension trial, RP103-04.
- pharmacokinetic results in *Section 12.3 Pharmacokinetics* from a completed single-dose, open-label renal impairment study RP103 16-001.

CONTENT OF LABELING

As soon as possible, but no later than 14 days from the date of this letter, submit the content of labeling [21 CFR 314.50(l)] in structured product labeling (SPL) format using the FDA automated drug registration and listing system (eLIST), as described at FDA.gov.[1] Content of labeling must be identical to the enclosed labeling (text for the Prescribing Information (PI), Patient Information (PPI) and Instructions for Use (IFU)), with the editorial changes in the PI (removed vertical lines in the left margin in Sections 2.2, 2.3, 2.4, 2.5, and 2.6 of the full prescribing information), addition of any labeling changes in pending "Changes Being Effected" (CBE) supplements, as well as annual reportable changes not included in the enclosed labeling.

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]

[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.

The SPL will be accessible from publicly available labeling repositories.

Also within 14 days, amend all pending supplemental applications that include labeling changes for this NDA, including CBE supplements for which FDA has not yet issued an action letter, with the content of labeling [21 CFR 314.50(l)(1)(i)] 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 NDA (21 CFR 314.80 and 314.81).

If you have any questions, call LCDR Hong Vu, Regulatory Project Manager, at (301) 796-7401.

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:
Content of Labeling
Prescribing Information
Patient Information
Instructions for Use

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/

DRAGOS G ROMAN
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