

BLA 761047/S-03

SUPPLEMENT APPROVAL

Ultragenyx Pharmaceutical Inc.
Attention: Annette Hillebrand, PhD, RAC
Director, Global Regulatory Affairs
60 Leveroni Court
Novato, CA 94949

Dear Dr. Hillebrand:

Please refer to your supplemental biologics license application (sBLA), dated July 29, 2019, submitted under section 351(a) of the Public Health Service Act for MEPSEVII (vestronidase alfa-vjbc) injection.

This Prior Approval supplemental biologics application provides for updates to section 8.1 Pregnancy in the Prescribing Information to reflect the results in the final report for study UX003-PC010, a pre- and post-natal development study in rats.

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 (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.

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>.

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.

FULFILLMENT OF POSTMARKETING REQUIREMENT

We have received your submission dated July 29, 2019, containing the final report for the following postmarketing requirement:

3271-2 A pre- and post-natal developmental study in rats to assess the effects of Mepsevii (vestronidase alfa-vjvk) on pre- and post-natal development. The study should be designed to detect adverse effects on the pregnant/lactating female rat and on the development of conceptus and offspring from implantation through weaning. The dose levels used in the pre- and post-natal developmental study should provide adequate margins of exposure for the clinical dose.

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

We remind you that there are postmarketing requirements and postmarketing commitments listed in the November 15, 2017, approval letter and February 27, 2018, new postmarket commitments 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, call Jenny Doan, Regulatory Project Manager, at (301) 796-1023.

Sincerely,

{See appended electronic signature page}

Dragos Roman, M.D.
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

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
12/06/2019 05:12:51 PM