Our STN: BL 125426/200

NOTIFICATION OF NON-COMPLIANCE WITH PREA
January 25, 2022

Medexus Pharma, Inc.
Attention: Khaled M Mohamed
29 N. Wacker Dr., Suite 704
Chicago, IL 60606

Dear Mr. Mohamed:

Please refer to your Biologics License Application (BLA) submitted under section 351(a) of the Public Health Service Act for Coagulation Factor IX (Recombinant).

The Agency has determined that you have failed to meet the postmarketing requirement (PMR) of the Pediatric Research Equity Act (PREA) for this application because you have not yet submitted your pediatric assessment for PMR 1, which was deferred until December 14, 2021.

- STN 125426/0 BLA Approval on May 14, 2015 with PMR 1:

  1. Deferred pediatric study IB1001-02 under PREA for the treatment of hemophilia B in pediatric patients ages 0 to 12. As appropriate, pediatric data from ongoing study IB1001-01 should be included in the submission.

     Final Protocol Submission: By July 31, 2015, you will submit a protocol revision that reopens pediatric study IB1001-02 to enrollment, in order to fulfill the commitment in your pediatric deferral request dated June 17, 2014.

     Study Completion Date: September 30, 2017

     Final Report Submission: December 31, 2017

- STN 125426/31 Release From Postmarketing Requirement/New Postmarketing Requirement on January 20, 2016:

  Release from PMR 1:

  1. Deferred pediatric study IB1001-02 under PREA for the treatment of hemophilia B in pediatric patients ages 0 to 12. As appropriate, pediatric data from ongoing study IB1001-01 should be included in the submission.

Final Protocol Submission: By July 31, 2015, you will submit a protocol revision that reopens pediatric study IB1001-02 to enrollment, in order to fulfill the commitment in your pediatric deferral request dated June 17, 2014.

Study Completion Date: September 30, 2017

Final Report Submission: December 31, 2017

New PMR 1:

1. Deferred pediatric study IB1001-02B under PREA for the treatment of hemophilia B in pediatric patients ages 0-12.

   Final Protocol Submission: Received on November 6, 2015

   Study Completion Date: June 29, 2018

   Final Report Submission: December 14, 2018

- STN 125426/154 Deferral Extension Granted on October 17, 2018:

  1. Deferred pediatric study IB1001-02B under PREA for the treatment of hemophilia B in pediatric patients ages 0-12.

     Original Final Report Submission: December 14, 2018

     New Final Report Submission: December 14, 2021

Under the provisions of title V, section 505, of the Food and Drug Administration Safety and Innovation Act of 2012 (FDASIA), you must respond in writing within 45 calendar days of the date of this letter. Your response should include the reason(s) for the delayed pediatric assessment and a date by which you expect to submit the assessment.

You may also include a request for a deferral extension, if applicable, which should be identified as a "**DEFERRAL EXTENSION REQUESTED**" in your response.

In accordance with FDASIA, FDA will post this letter and your response on the website located at https://www.fda.gov/AboutFDA/CentersOffices/OfficeofMedicalProductsandTobacco/CBER/ucm448393.htm with redactions for any trade secrets and confidential commercial information 60 calendar days from the date of this letter.

Please submit your response to this letter within 45 days to this STN BL 125426. Please identify your response to this letter as a "**RESPONSE TO PREA NON-COMPLIANCE LETTER.**" To facilitate our review, submit a cross-reference letter to the IND to which your protocol has been submitted.

If you have any questions, please contact the Regulatory Project Manager, Catherine Tran, at catherine.tran@fda.hhs.gov.

Sincerely,

Tejashri Purohit-Sheth, MD
Director
Division of Clinical Evaluation and
Pharmacology/Toxicology
Office of Tissues and Advanced Therapies
Center for Biologics Evaluation and Research