Our STN: BL 125426/223

SUPPLEMENT APPROVAL
PMR FULFILLED
March 22, 2024

Medexus Pharma, Inc.
Attention: Khaled Mohamed
29 N Wacker Ave
Chicago, IL 60606

Dear Khaled Mohamed:

We have approved your request received May 24, 2023, to supplement your Biologics License Application (BLA) submitted under section 351(a) of the Public Health Service Act for Coagulation Factor IX (Recombinant) to fulfill the required pediatric assessment for coagulation factor IX (recombinant) per STN BL 125426/222, and to expand the label to include pediatric patients <12 years of age for the treatment of Hemophilia B based on the data derived from the PMR study.

The review of this supplement was associated with the following National Clinical Trial (NCT) number: 03855280.

LABELING

Under 21 CFR 201.57(c)(18), patient labeling must be referenced in section 17 PATIENT COUNSELING INFORMATION. Patient labeling must be available and may either be reprinted immediately following the full prescribing information of the package insert or accompany the prescription product labeling.

We hereby approve the draft content of labeling Package Insert and Patient Package Insert submitted under amendment 8, dated March 11, 2024.

CONTENT OF LABELING

As soon as possible, but no later than 14 days from the date of this letter, please submit the final content of labeling (21 CFR 601.14) in Structured Product Labeling (SPL) format via the FDA automated drug registration and listing system, (eLIST) as described at http://www.fda.gov/ForIndustry/DataStandards/StructuredProductLabeling/default.htm. Content of labeling must be identical to the Package Insert and Patient Package Insert submitted on March 11, 2024. 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* at

http://www.fda.gov/downloads/Drugs/GuidanceComplianceRegulatoryInformation/Guidances/UCM072392.pdf.

The SPL will be accessible via publicly available labeling repositories.

All final labeling should be submitted as Product Correspondence to this BLA, STN BL 125426 at the time of use and include implementation information on Form FDA 356h.

ADVERTISING AND PROMOTIONAL LABELING

You may submit two draft copies of the proposed introductory advertising and promotional labeling with Form FDA 2253 to the Advertising and Promotional Labeling Branch at the following address:

Food and Drug Administration
Center for Biologics Evaluation and Research
Document Control Center
10903 New Hampshire Ave.
WO71–G112
Silver Spring, MD 20993-0002

You must submit copies of your final advertising and promotional labeling at the time of initial dissemination or publication, accompanied by Form FDA 2253 (21 CFR 601.12(f)(4)).

All promotional claims must be consistent with and not contrary to approved labeling. You should not make a comparative promotional claim or claim of superiority over other products unless you have substantial evidence or substantial clinical experience to support such claims (21 CFR 202.1(e)(6)).

For each pending supplemental application for this BLA that includes proposed revised labeling, please submit an amendment to update the proposed revised labeling with the changes approved today.

FULFILLED POSTMARKETING REQUIREMENT/COMMITMENTS

This submission fulfills your postmarketing requirement PMR #1 identified in the March 23, 2023 approval letter for BLA STN BL 125426/222 for Coagulation Factor IX (Recombinant). The requirement addressed in this submission is as follows:

1. Deferred pediatric study ib1001-02b under PREA for the treatment of Hemophilia B in pediatric patients ages 0<12 years of age

   Final Report Submission: December 14, 2021

   New Final Report Submission: June 30, 2023

PEDIATRIC REQUIREMENTS

Under the Pediatric Research Equity Act (PREA) (21 U.S.C. 355c), all applications for new active ingredients, 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 in pediatric patients unless this requirement is waived, deferred, or inapplicable.

We note that you have fulfilled the pediatric study requirement for all relevant pediatric age groups for this application.

We will include information contained in the above-referenced supplement in your BLA file.

Sincerely,

Lola Fashoyin-Aje, MD, MPH
Acting Director
Division of Clinical Evaluation Hematology
Office of Clinical Evaluation
Office of Therapeutic Products
Center for Biologics Evaluation and Research