Our STN: BL 103677/6937

PARTIAL RESCISSION OF APPROVAL
February 17, 2021

Wyeth Pharmaceuticals LLC
Attention: Nicole Parker, PhD
Pfizer
235 East 42nd Street
New York, NY 10017-7555

Dear Dr. Parker:

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

We also refer to FDA's letter dated June 26, 2020, for your BLA supplement STN BL 103677/6512 to add an indication for routine prophylaxis indication in adult and children with hemophilia B (congenital factor IX deficiency or Christmas disease) which was approved.

Upon review of Agency records, we have determined that FDA erred in approving the indication for routine prophylaxis in children with hemophilia B (congenital factor IX deficiency or Christmas disease) on June 26, 2020. As noted below, your product was not eligible to be approved for this particular indication at that time because another licensed product, Baxalta US Inc.'s product, RIXUBIS, Coagulation Factor IX (Recombinant)] (BLA 125446) ("RIXUBIS"), had orphan-drug exclusivity blocking such approval. Please note that the indication for routine prophylaxis in **adults** with hemophilia B (congenital factor IX deficiency or Christmas disease) is not at issue, and the June 26, 2020 approval for the adult indication remains in effect.

The Orphan Drug provisions of the FD&C Act, 21 U.S.C. §§ 360aa *et seq.*, provide for, among other things, seven years of exclusivity that blocks approval of another applicant's application for the same drug for the same indication. The orphan-drug exclusivity for RIXUBIS is in effect until September 12, 2021. Therefore, to the extent that your supplement BLA 103677/6512 added an indication for routine prophylaxis in children with hemophilia B (congenital factor IX deficiency or Christmas disease), it should not have been approved, and is rescinded.

FDA is correcting its error to make clear that the approval of your supplement for BLA 103677/6512 only covers the indication for routine prophylaxis in adults with hemophilia B (congenital factor IX deficiency or Christmas disease). Therefore, your labeling needs to be revised to remove the exclusivity protected indication [i.e., routine prophylaxis in

children with hemophilia B (congenital factor IX deficiency or Christmas disease)]. Attached is revised draft labeling for BENEFIX, Coagulation Factor IX (Recombinant). We note that any other applications for Coagulation Factor IX (Recombinant) for routine prophylaxis in children with hemophilia B (congenital factor IX deficiency or Christmas disease) currently approved or pending approval prior to the expiration of the above-stated exclusivity on September 12, 2021 will also need to have their labeling updated in accordance with the attached model labeling.

Please submit updated labeling as an amendment to STN BL 103677/6937 no later than February 23, 2021. Submit labeling exactly as specified above as a Supplement - Changes Being Effected, and incorporate all revisions since the last approval of the prescribing information. To facilitate review of your submission, provide a highlighted or marked-up copy that shows all changes, as well as a clean Microsoft Word version. The marked-up copy should also include annotations that support all proposed changes, including annual reportable changes. Your supplement must include updated content of labeling 21 CFR 601.14(b)] in structured product labeling (SPL) format as described at FDA.gov.

If you have any questions, please contact Edward Thompson at edward.thompson@fda.hhs.gov.

Sincerely,

Tejashri Purohit-Sheth, MD
Director
Division of Clinical Evaluation
and Pharmacology/Toxicology
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