

Mid-Cycle Communication Telecon

Application type: Original BLA

Tracking number: STN 125577/0

Product name: von Willebrand Factor (Recombinant)

Proposed Indication: For the prevention and treatment of bleeding episodes in adults (age 18 years and older) diagnosed with von Willebrand disease

Applicant: Baxter Healthcare Corporation

Meeting date & time: 11 June 2015, Thursday at 1:00 PM

Committee Chair: Chava Kimchi-Sarfaty, PhD

RPM: Cherie Ward-Peralta, MS

CBER Review Team Attendees:

Chava Kimchi-Sarfaty, PhD, CBER/OBRR/DHRR/LH
Cherie Ward-Peralta, MS, RPM, CBER/OBRR

Other Attendees:

Christopher Sese, Independent Assessor, Eastern Research Group

Baxter Attendees:

Mehrshid Alai, Senior Director, Global Regulatory Affairs
Bita Badiei, Associate Director, Global Regulatory Affairs
Erik Bjornson, Director, Global Regulatory Affairs
Max Fernandez, Senior Manager, Global Regulatory Affairs
Nikhil Mehta, Vice President, Global Regulatory Affairs
Bruce Ewenstein, Vice President, Clinical Strategy, Hemophilia
Ortrun Obermann-Slupetzky, Manager, Clinical Research, Hematology
Anne Prener, Vice President, Clinical Research, Hematology
Isabella Presch, Medical Director, Hematology
Natalia Seibel, Senior Quality Product Owner
Robert Weiss, Director, Quality
Paulien Groll, Director, Quality
Peter Leidenmuehler, Manager, Non-Clinical Development
Sandor Fritsch, Senior Director, Global Biostatistics

Discussion Summary:

1. No significant issues or major deficiencies have been identified by the review committee to date.

No further discussion

2. At this time, there are no major safety concerns. The current thinking of the review committee is that a *Risk Evaluation and Mitigation Strategy* (REMS) is not required.

No further discussion

3. With regard to risk management, routine pharmacovigilance is recommended.

No further discussion

4. The current thinking of the review committee is that this BLA will not be presented at the *Blood Products Advisory Committee* meeting.

No further discussion

5. Information requests (IRs) sent but responses from Baxter have not been received:

- FDA sent an IR about depyrogenation, lyophilization, container closure, visual inspection for the final drug product, and diluents on 22 May 2015; and is expecting Baxter's response by 22 June 2015.
- FDA sent an IR about clinical/package insert, clinical pharmacology, and reagents and lots on 3 June 2015; and is expecting Baxter's response by 19 June 2015.

Discussion:

FDA informed Baxter that additional CMC IRs will be sent out in the next 6-8 weeks.

Baxter informed FDA that there are only (b) (4) lots available to respond to one of the questions in the June 3, 2015 IR. FDA stated that data from (b) (4) lots within the proposed shelf-life would be fine to respond to our question.

6. Please note that the review is ongoing and additional information may be requested as the need arises.
7. The *Late-Cycle Meeting* is scheduled on Thursday, 3 September 2015 from 9:30 AM to 11:30 AM, and the format of the meeting will be determined at a later date.

Discussion:

The format of the meeting will be dependent on how the review progresses, and is pending internal discussion at FDA. FDA has reserved a room appropriate for a face-to-face meeting, and will finalize the format upon discussion with Baxter.

8. The action due date for this BLA is 19 December 2015.

No further discussion

Additional Discussions:

9. Baxter asked for an update on the status of the review on the non-clinical sections of the BLA. FDA responded that the review of the non-clinical data is still ongoing.
10. Baxter asked for an update on the status of the review of the responses to the 483 observations at the (b) (4) facilities. FDA stated that the review is still ongoing; and Baxter can expect a response before the additional CMC IRs are sent.
11. Baxter asked FDA to share the Agency's current thinking regarding the need for a pre-license inspection (PLI) of the Thousand Oaks facility. FDA stated that the review committee does not see a need at this time to conduct a PLI of the Thousand Oaks facility.

End