

MEMORANDUM

DEPARTMENT OF HEALTH AND HUMAN SERVICES
Public Health Service
Food and Drug Administration
Center for Biologics Evaluation and Research

DATE August 28, 2015

FROM Colonious King, Bioresearch Monitoring Branch
Division of Inspections and Surveillance
Office of Compliance and Biologics Quality
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THROUGH Patricia Holobaugh, Chief, Bioresearch Monitoring Branch

THROUGH Gilliam Conley, Director, Division of Inspections and Surveillance

TO Chava Kimchi-Sarfaty Chair, Review Committee
Cherie Ward-Peralta RPM
Victor Baum Clinical Reviewer

SUBJECT Bioresearch Monitoring Discipline Review Memo
BLA/STN: 125577/0
IND: 13657
Sponsor: Baxter Healthcare Corporation
Product: Recombinant von Willebrand factor: recombinant factor VIII
(rVWF:rFVIII); VONVENDI

FINAL SUMMARY STATEMENT:

The Bioresearch Monitoring inspection of two clinical investigators did not reveal problems that impact the data submitted in this Biologics Licensing Application (BLA).

BACKGROUND

Two clinical investigators were inspected in support of the BLA and were conducted in accordance with FDA's Compliance Program Guidance Manual (CPGM) 7348.811, Inspection Program for Clinical Investigators. The inspection assignment included specific questions related to the study protocols and verification of the study data on safety and efficacy endpoints submitted by the sponsor in the BLA. The completed inspections conducted at two clinical sites for data verification represented 10% of all the subjects studied for this new BLA application. The data audit portion of the inspections focused on the verification of the study data on safety and efficacy endpoints submitted by the sponsor in the BLA for 100% of the enrollees at site 02 and site 06.

PROTOCOL AUDITED

A Phase 3 clinical study to determine the pharmacokinetics, safety and efficacy of rVWF: rFVIII and rVWF in the treatment of bleeding episodes in subjects diagnosed with von Willebrand disease. (Protocol 071001)

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The table below summarizes the inspection results:

Site Number	Study Site	Location	Enrolled Subjects	483 Issued	Classification
02	Blood Center of Wisconsin Comprehensive center for Bleeding Disorders	Milwaukee, Wisconsin	4	No	NAI
06	Rochester General Hospital Mary M. Gooley Hemophilia Center	Rochester, New York	1	No	NAI

NAI = No Action Indicated

FINANCIAL DISCLOSURE

The Clinical Investigator Compliance Program directs the FDA investigator to ask the clinical investigator if and when s/he disclosed information about her/his financial interests to the sponsor and/or interests of any sub-investigators, spouse(s) and dependent children including if and when the information was updated. The inspected study site had a copy of the financial disclosure forms on hand for the clinical investigator and sub-investigators.

INSPECTIONAL FINDINGS

Sponsor/Monitor Issues

There were no sponsor/monitor issued identified at the study sites audited.

Clinical Investigator (CI) Study Site Issues

Study Site 02 and 06: A Form FDA 483 was not issued at close of these inspections and the inspection were classified as NAI. A review was conducted of testing records, regulatory binders, study specific standard operating procedures, and general study conduct. In addition, source documents were reviewed and the information was compared to the data tables submitted by the sponsor in the application. No discrepancy was found between source documents at the site and the data submitted by the sponsor in the application.

BIMO ADMINISTRATIVE FOLLOW-UP

Information letters were issued for the study sites inspected.

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Please contact me should you have any questions about this memo or any aspect of Bioresearch Monitoring.

Colonious King
Consumer Safety Officer

Distribution

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EDR	STN 125577/0
Chava Kimchi-Sarfaty	Chair
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Victor Baum	Clinical Reviewer
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Draft: King: 08/27/15

Reveiwed:Holobaugh:08/28/15