FOR IMMEDIATE RELEASE

Octapharma's Prothrombin Complex Concentrate, Balfaxar®, Receives FDA Approval For Warfarin Reversal in Urgent Surgery & Invasive Procedures

PARAMUS, N.J., July 26, 2023 – Octapharma USA today announced that Balfaxar® (prothrombin complex concentrate, human-lans; marketed in Europe and Canada as octaplex®) has received U.S. Food and Drug Administration (FDA) approval for the urgent reversal of acquired coagulation factor deficiency induced by vitamin K antagonist (VKA, e.g., warfarin) therapy in adult patients with need for urgent surgery or invasive procedures.

Balfaxar® helps restore blood coagulation by replenishing the levels of clotting factors that are deficient due to warfarin therapy. Balfaxar® is a non-activated four factor prothrombin complex concentrate (4F-PCC) containing vitamin K-dependent factors: Factor II (prothrombin), Factor VII, Factor IX and Factor X, as well as antithrombotic Proteins C and S.

The FDA approval is supported by the clinical trial LEX-209 (ClinicalTrials.gov Identifier: NCT02740335), which compared the efficacy and safety of Balfaxar® head-to-head with a control 4F-PCC (Kcentra®). The Phase III, randomized, double-blind, multicenter study was performed at 24 sites in the U.S. and Europe and randomized 208 patients to Balfaxar® (N=105) or control 4F-PCC (N=103).

"Balfaxar® met the primary endpoint of hemostatic efficacy and was non-inferior to the comparator, Kcentra®, in patients on a vitamin K antagonist undergoing urgent surgery with significant bleeding risk," said LEX-209 Principal Investigator Ravi Sarode, M.D. "The primary objective was met at the prespecified interim analysis and the study was stopped due to statistically significant efficacy results indicating that Balfaxar® was non-inferior to Kcentra®. Balfaxar® demonstrated effective hemostasis in 94.6% of patients versus 93.5% of patients for Kcentra®. International Normalized Ratio (INR) reductions and vitamin K dependent coagulation factor increases supported the primary endpoint and PCC dosing and duration of infusion were also similar. The safety profile was similar between treatment arms and consistent with previous studies."[1]

More than 2.4 million U.S. patients are prescribed warfarin to prevent blood clots from forming following a heart attack, heart valve surgery, stroke, deep vein thrombosis/pulmonary embolism, or for certain types of irregular heartbeat (atrial fibrillation).[2] The main side effect of warfarin is an increased risk of bleeding particularly for patients undergoing urgent surgery or invasive procedures.[3]

"The FDA approval of Balfaxar® establishes a new therapy for medical providers when their patients need a 4F-PCC product," said Octapharma USA President Flemming Nielsen. "Octapharma is committed to providing patients with life-saving and life-enhancing therapies for critical care medicine. We are confident Balfaxar® will be a welcomed treatment for physicians who need to quickly restore patients' coagulation."

Balfaxar®, a lyophilized powder for reconstitution, will be provided with sterile water for injection and the new transfer device, nextaro® The transfer device includes an optimized vial housing that enables precentering of the vial during mounting, and optimized contamination protection with two integrated filters. Nextaro® was preferred by healthcare professionals versus a widely used, competitive transfer device in an Octapharma user preference study.[4]

To study Balfaxar® further in other clinical scenarios, Octapharma is recruiting for two additional Phase III studies:

- LEX-210: Study of OCTAPLEX in Patients With Acute Major Bleeding on DOAC Therapy With Factor Xa Inhibitor (ClinicalTrials.gov Identifier: NCT04867837)
- LEX-211: Active-control Randomised Trial Comparing 4-factor Prothrombin Complex Concentrate With Frozen Plasma in Cardiac Surgery (ClinicalTrials.gov Identifier: NCT05523297)

For more information on LEX-210 and LEX-211, please visit ClinicalTrials.gov.

Kcentra® is a registered trademark of CSL Behring GmbH.

Nextaro® is a registered trademark of sfm medical devices GmbH.

About Balfaxar®

Balfaxar® (prothrombin complex concentrate, human-lans) is a blood coagulation factor replacement product indicated for the urgent reversal of acquired coagulation factor deficiency induced by Vitamin K antagonist (VKA, e.g., warfarin) therapy in adult patients with need for an urgent surgery/invasive procedure.

WARNING: ARTERIAL and VENOUS THROMBOEMBOLIC COMPLICATIONS

Patients being treated with Vitamin K antagonists (VKA) therapy have underlying disease states that predispose them to thromboembolic events. Potential benefits of reversing VKA should be weighed against the potential risks of thromboembolic events, especially in patients with the history of a thromboembolic event. Resumption of anticoagulation should be carefully considered as soon as the risk of thromboembolic events outweighs the risk of acute bleeding.

- Both fatal and non-fatal arterial and venous thromboembolic complications have been reported with Balfaxar® in clinical trials and post marketing surveillance. Monitor patients receiving Balfaxar® for signs and symptoms of thromboembolic events.
- Balfaxar® may not be suitable in patients with thromboembolic events in the prior 3 months.

For complete boxed warning and full prescribing information, please visit Balfaxar.com/pi.

About the Octapharma Group

Headquartered in Lachen, Switzerland, Octapharma is one of the largest human protein manufacturers in the world, developing and producing human proteins from human plasma and human cell lines.

Octapharma employs more than 11,000 people worldwide to support the treatment of patients in 118 countries with products across three therapeutic areas: Hematology, Immunotherapy and Critical Care.

Octapharma has seven R&D sites and five state-of-the-art manufacturing facilities in Austria, France, Germany and Sweden, and operates more than 190 plasma donation centers across Europe and the US. The company's American subsidiary, Octapharma USA, is located in Paramus, N.J. For more information, please visit octapharmausa.com.

REFERENCES

1 - Ravi Sarode, Joshua N. Goldstein, Gregory Simonian, Truman J. Milling Jr; A Phase 3, Prospective, Randomized, Double-Blind, Multicenter, Non-Inferiority Study Comparing Two Four-Factor Prothrombin Complex Concentrates for Reversal of Vitamin K Antagonist-Induced Anticoagulation in Patients Needing Urgent Surgery with Significant Bleeding Risk. Blood 2022; 140 (Supplement 1): 352–353. doi: https://doi.org/10.1182/blood-2022-168890

2 - ClinCalc.com, Warfarin: Drug Usage Statistics, United States, 2013 - 2020, accessed June 19, 2023.

3 - Mayo Clinic, Warfarin side effects: Watch for interactions, accessed June 19, 2023.

4 - Data on file, Octapharma USA, Oct. 22, 2018.

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