



Chugai's Hemlibra® Gains Positive CHMP Opinion in Severe Hemophilia A without Inhibitors

TOKYO, February 4, 2019 -- [Chugai Pharmaceutical Co., Ltd.](#) (TOKYO: 4519) announced today that Roche has received notification that the EU Committee for Medicinal Products for Human Use (CHMP) adopted a positive opinion for Hemlibra®, a treatment for hemophilia A created by Chugai, for routine prophylaxis of bleeding episodes in adults and children with severe hemophilia A without factor VIII inhibitors, administered once weekly, every two weeks, or every four weeks. The CHMP has also adopted a positive opinion for additional dosing options of every two weeks or every four weeks in adults and children with hemophilia A with factor VIII inhibitors.

“We are thrilled that Hemlibra is expected to be approved shortly for people with severe hemophilia A without inhibitors in the Europe Union (EU). Also, I’m very pleased that people in the EU with hemophilia A will soon be offered multiple options of Hemlibra’s dosing interval regardless of their inhibitor expression,” said Chugai’s Executive Vice President, Co-Head of Project & Lifecycle Management Unit, Dr. Osamu Okuda. “Hemlibra is co-promoted by Chugai and Roche in Germany, France, and the United Kingdom, as is the case with the anti-rheumatic agent RoActemra®. We are committed to pursue our efforts in collaboration with Roche so that Hemlibra may further contribute to the treatment of hemophilia A.”

This positive opinion is based on results from two Phase III studies HAVEN 3 (NCT02847637) and HAVEN 4 (NCT03020160), conducted jointly with Roche and Genentech. HAVEN 3 study was conducted to evaluate the reduction of bleed rate of Hemlibra subcutaneous injection once a week and once every two weeks in people with hemophilia A (12 years of age or older) without inhibitors to factor VIII. HAVEN 4 study was conducted to evaluate efficacy, safety, and pharmacokinetics of Hemlibra subcutaneous injection every four weeks in people with hemophilia A (12 years of age or older), with and without inhibitors to factor VIII.

In Japan, Chugai obtained regulatory approval for Hemlibra from the Ministry of Health, Labour and Welfare in December 2018 for an additional indication of prophylactic treatment for people with hemophilia A without inhibitors to factor VIII, as well as for additional dosage and administration as a biweekly or every four-week treatment for people with hemophilia A with inhibitors to factor VIII.

References

Chugai’s HEMLIBRA® Receives Regulatory Approval from U.S. FDA for Hemophilia A without Inhibitors

Press release issued on October 5, 2018

https://www.chugai-pharm.co.jp/english/news/detail/20181005100000_553.html

Chugai's HEMLIBRA® Subcutaneous Injection Receives Approval for Hemophilia A without Inhibitors and Extension of Dosing Interval -- HEMLIBRA is now available for Hemophilia A, regardless of inhibitor status –

Press release issued on December 21, 2018

https://www.chugai-pharm.co.jp/english/news/detail/20181221153002_580.html

About Severe Hemophilia A

People with severe hemophilia A is defined as the condition with less than 1 % of factor VIII levels. Hemophilia A affects around 320,000 people worldwide, ^{1,2} approximately 50-60% of whom are expected to have a severe form of the disorder³.

1) WFH. Guidelines for the management of haemophilia. 2012 [Internet; cited 2018 July]. Available from:

<http://www1.wfh.org/publications/files/pdf-1472.pdf>. (as of February 1, 2019)

2) Berntorp E, Shapiro AD. Modern haemophilia care. The Lancet 2012; 370:1447-1456.

3) Marder VJ, et al. Hemostasis and Thrombosis. Basic Principles and Clinical Practice. 6th Edition, 2013. Milwaukee, Wisconsin. Lippincott Williams and Wilkin.

About Chugai

Chugai Pharmaceutical is one of Japan's leading research-based pharmaceutical companies with strengths in biotechnology products. Chugai, based in Tokyo, specializes in prescription pharmaceuticals and is listed on the 1st section of the Tokyo Stock Exchange. As an important member of the Roche Group, Chugai is actively involved in R&D activities in Japan and abroad. Specifically, Chugai is working to develop innovative products which may satisfy the unmet medical needs, mainly focusing on the oncology area.

Additional information is available on the internet at <https://www.chugai-pharm.co.jp/english>.

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