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Chugai's HEMLIBRA® Receives Breakthrough Therapy Designation from U.S. FDA for Hemophilia A without Factor VIII Inhibitors

—The Sixth Designation for Chugai Originated Drug—

TOKYO, April 17, 2017 -- Chugai Pharmaceutical Co., Ltd. (TOKYO: 4519) announced today that the U.S. Food and Drug Administration (FDA) has granted Breakthrough Therapy Designation for its anti-coagulation factor IXa/X humanized bispecific monoclonal antibody / coagulation factor VIII substitute, "HEMLIBRA®" [US generic name: emicizumab-kxwh] for people with hemophilia A without factor VIII inhibitors. Development and distribution of the drug in the US is conducted by Genentech, a member of Roche Group.

"We are thrilled that HEMLIBRA has been granted its second Breakthrough Therapy Designation," said Chugai's Executive Vice President, Co-Head of Project & Lifecycle Management Unit, Dr. Yasushi Ito. "This will allow us to expedite potential delivery of this new therapy we created to people with hemophilia A without inhibitors in the US following the previous designation for inhibitors. We continue to work closely with Genentech to enable this line extension as soon as possible."

This designation is based on the global phase III HAVEN 3 (NCT02847637) study evaluating 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.

Hemophilia A is a disease presenting repeated severe bleeding symptoms. In this disease, the blood coagulation reaction does not proceed normally due to the deficiency or functional disorder of coagulation factor VIII. For people with hemophilia A without inhibitors, regular factor VIII replacement therapy has been widely used to prevent bleeding. HEMLIBRA is a bispecific monoclonal antibody, which was developed using Chugai's proprietary antibody engineering technologies. The drug is designed to bind factor IXa and factor X. In doing so, HEMLIBRA provides the cofactor function of factor VIII in people with hemophilia A, who either lack or have impaired coagulation function of factor VIII[1, 2]. In the HAVEN 3 study, a statistically significant reduction in the frequency of bleeding episodes was observed with HEMLIBRA. With the convenience of subcutaneous administration and the lower frequency of administration, it is expected to be a new treatment option for hemophilia A.

This is the sixth Breakthrough Therapy Designation received for three drugs created by Chugai: ALECENSA® (ALK-positive non-small cell lung cancer with disease progression on crizotinib, and first line treatment for ALK-positive non-small cell lung cancer), ACTEMRA® (systemic sclerosis and giant cell arteritis), and HEMLIBRA (prophylactic treatment for patients 12 years or older with hemophilia A with factor VIII inhibitors).

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References

1. Kitazawa, et al. Nature Medicine 2012; 18(10): 1570
2. Sampei, et al. PLoS ONE 2013; 8: e57479

About Breakthrough Therapy

Breakthrough Therapy Designation was adopted as part of the FDA Safety and Innovation Act (FDASIA) enacted in July 2012 aiming at expediting the development and review of drugs for the treatment of severe or life-threatening diseases or symptoms. In order to grant Breakthrough Therapy Designation, preliminary clinical evidence is required demonstrating that the drug may have substantial improvement on at least one clinically significant endpoint over existing therapies. Breakthrough Therapy Designation includes the features of a Fast Track designation, with the addition of intensive guidance on efficient drug development as well organizational commitment from FDA.

Main approval status of the drug

US: In November 2017, the drug (US product name: HEMLIBRA®; Genentech) was approved by the U.S. Food and Drug Administration and was marketed for “routine prophylaxis to prevent or reduce the frequency of bleeding episodes in adult and pediatric patients with hemophilia A (congenital factor VIII deficiency) with factor VIII inhibitors.”

EU: In Europe, the drug (EU product name: HEMLIBRA®; Roche) obtained regulatory approval from the European Commission and was marketed for routine prophylaxis of bleeding episodes in people with hemophilia A with factor VIII inhibitors in February 2018.

Japan: The Ministry of Health, Labour and Welfare has approved HEMLIBRA for routine prophylaxis to prevent or reduce the frequency of bleeding episodes in patients with congenital factor VIII deficiency (hemophilia A) with factor VIII inhibitors in March 2018.

About the results of HAVEN 3 study

Press release issued on November 20, 2017
https://www.chugai-pharm.co.jp/english/news/detail/20171120151500.html

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.
In Japan, Chugai’s research facilities in Gotemba and Kamakura are collaborating to develop new pharmaceuticals and laboratories in Ukima are conducting research for technology

development for industrial production. Overseas, Chugai Pharmabody Research based in Singapore is engaged in research focusing on the generation of novel antibody drugs by utilizing Chugai's proprietary innovative antibody engineering technologies. Chugai Pharma USA and Chugai Pharma Europe are engaged in clinical development activities in the United States and Europe.
The consolidated revenue in 2017 of Chugai totaled 534.2 billion yen and the operating income was 103.2 billion yen (IFRS Core basis).
Additional information is available on the internet at https://www.chugai-pharm.co.jp/english.

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