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Chugai's HEMLIBRA® Receives the World's First Regulatory Approval from FDA for Hemophilia A with Inhibitors

TOKYO, November 17, 2017 -- Chugai Pharmaceutical Co., Ltd. (TOKYO: 4519) announced today that the U.S. Food and Drug Administration (FDA) has approved the bispecific antibody emicizumab (US product name: HEMLIBRA®) 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. This represents the first regulatory approval for HEMLIBRA® around the world. The US Biologics License Application (BLA) was submitted by Genentech, a member of Roche Group.

"We are pleased that HEMLIBRA, a biopharmaceutical created by Chugai scientists based on their exceptional idea, has received its first regulatory approval," said Chugai's President & COO, Tatsuro Kosaka. "HEMLIBRA is a first in class biopharmaceutical which is an advancement on the current limited treatment options available for patients with hemophilia A with inhibitors. We anticipate that patients will receive significant potential benefits from HEMLIBRA, which enables once-weekly subcutaneous injection as well as reduction of the bleeding risk."

This regulatory approval is based on the data from two clinical studies, the results of HAVEN 1 study (NCT02622321) and the interim analysis of HAVEN 2 study (NCT02795767) in patients with hemophilia A with factor VIII inhibitors. HAVEN 1 included adult and adolescent patients and HAVEN 2 included pediatric patients, and were both conducted under a collaboration between Chugai, Roche and Genentech. In the HAVEN 1 study, adults and adolescents with hemophilia A with inhibitors who received HEMLIBRA prophylaxis (n=35) had a statistically significant and clinically meaningful 87% (95% CI: 72.3-94.3, $p<0.0001$) reduction in treated bleeds compared with those who received no prophylaxis (n=18). Interim results from the HAVEN 2 study, pediatric patients with hemophilia A with inhibitors showed that 87% of patients (95% CI: 66.4-97.2) who received HEMLIBRA prophylaxis (n=23) experienced zero treated bleeds. The most common adverse events (AEs) occurring in 10% or more of people treated with HEMLIBRA in pooled studies were injection site reactions, headache and joint pain (arthralgia).

HEMLIBRA was reviewed by the FDA under Priority Review. It was granted Breakthrough Therapy Designation by the FDA in adults and adolescents with hemophilia A with inhibitors in September 2015. Outside the US, emicizumab has been filed in the EU in June 2017 and data from HAVEN 1 and HAVEN 2 are being reviewed under Accelerated Assessment by the European Medicines Agency. In Japan, the drug received orphan drug designation by the Ministry of Health, Labour and Welfare for the prevention and reduction of bleeding episodes in patients with congenital FVIII deficiency (hemophilia A) who developed inhibitors to FVIII in August 2016, and a new drug application was filed in July 2017.

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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 2016 of Chugai totaled 491.8 billion yen and the operating income was 80.6 billion yen (IFRS Core basis).

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

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