



Chugai's HEMLIBRA® Gains Positive CHMP Opinion in Hemophilia A with Inhibitors

TOKYO, January 29, 2018 -- [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® (emicizumab) for routine prophylaxis of bleeding episodes in people with hemophilia A with factor VIII inhibitors. The CHMP has recommended HEMLIBRA for use in all age groups.

“Following the regulatory approval from the U.S. Food and Drug Administration (FDA) in November 2017, we are thrilled that based on the CHMP opinion HEMLIBRA is expected to be approved by the European Commission in the near future,” said Chugai’s Senior Vice President, Head of Project & Lifecycle Management Unit, Dr. Yasushi Ito. “Existing treatments for people with hemophilia A with inhibitors need frequent intravenous injection every week, which remains a burden for both patients and their caregivers. HEMLIBRA has the benefit of enabling once-weekly subcutaneous (under the skin) injection. We are committed to deliver the drug to patients as early as possible in collaboration with Roche.”

This positive opinion is based on the two pivotal studies for hemophilia A with inhibitors: the results of HAVEN1 study (NCT02622321) for adolescents and adults, and the interim analysis of HAVEN2 study (NCT02795767) in children.

Emicizumab 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, emicizumab 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 November 2017, the drug (US product name: HEMLIBRA®; Genentech) was approved by the FDA 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.” An EU marketing authorization application was submitted in June 2017 and was reviewed under Accelerated Assessment by the European Medicines Agency. In Japan, emicizumab obtained an orphan drug designation in August 2016 from the Ministry of Health, Labour and Welfare for the prevention and reduction of bleeding episodes in patients with congenital factor VIII deficiency (hemophilia A) who developed inhibitors to factor VIII, followed by an application for regulatory approval filed in July 2017.

About the results of HAVEN 1 study and the interim analysis of HAVEN 2 study

Press release issued on June 26, 2017

<https://www.chugai-pharm.co.jp/english/news/detail/20170626140000.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 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>.

Trademarks used or mentioned in this release are protected by law.

References

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

Contact:

For Media

Chugai Pharmaceutical Co., Ltd.
Media Relations Group, Corporate Communications Dept.,
Koki Harada
Tel: +81-3-3273-0881
E-mail: pr@chugai-pharm.co.jp

For US media

Chugai Pharma USA Inc.
Paul Mignone
Tel: +1-908-516-1350
E-mail: pr@chugai-pharm.com

For European media

Chugai Pharma France SAS
Nathalie Leroy
Tel: +33-1-56-37-05-21
E-mail: pr@chugai.eu

For Taiwanese media

Chugai Pharma Taiwan Ltd.

Susan Chou

Tel: +886-2-2715-2000

E-mail: pr@chugai.com.tw

For Investors

Chugai Pharmaceutical Co., Ltd.

Investor Relations Group, Corporate Communications Dept.,

Toshiya Sasai

Tel: +81-3-3273-0554

E-mail: ir@chugai-pharm.co.jp