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New Data from Phase III HAVEN 6 Study Reinforce Favorable Safety and Efficacy Profile of Chugai's Hemlibra in People with Moderate or Mild Hemophilia A

- Hemlibra continues to demonstrate clinically meaningful bleed control, with 66.7% of participants with moderate or mild hemophilia A experiencing zero treated bleeds at 55.6 weeks median follow-up
- New data also reinforce Hemlibra's favorable safety profile with no new safety signals observed

TOKYO, July 12 2022 -- Chugai Pharmaceutical Co., Ltd. (TOKYO: 4519) announced results from the primary analysis of the phase III HAVEN 6 study, which show that Hemlibra® (emicizumab) continued to demonstrate a favorable safety profile and effective bleed control in people with moderate or mild hemophilia A, without factor VIII inhibitors.[1] The data was presented at the 30th International Society on Thrombosis and Haemostasis (ISTH) Annual Congress, on 11 July 2022 (local time) in London, United Kingdom. The data is planned to support a submission to the European Medicines Agency to update the label for Hemlibra to include non-severe hemophilia A patients.

"We are delighted that Hemlibra, a treatment created using Chugai's proprietary bispecific antibody technology, has demonstrated effective bleed control in moderate or mild hemophilia A following an interim analysis last year. The evidence of an evaluation period longer than one year provides significant value for patients using Hemlibra," said Dr. Osamu Okuda, Chugai's President and CEO. "High-quality evidence is essential to facilitate the use of innovation in healthcare settings. Chugai will continue cooperating with Roche in the generation of further evidence to maximize the value of Hemlibra as a treatment option for hemophilia A."

In addition to HAVEN 6, data from the CHESS II (Cost of Hemophilia across Europe: a Socioeconomic Survey-II) and CHESS PAEDs studies will also be presented at ISTH 2022. These data show most adults with moderate or mild hemophilia A and more than half of children with moderate hemophilia A may not receive preventative treatments. This can result in worsened clinical burden, as more than 30% of adults and approximately 40% of children with moderate hemophilia A who were not taking preventative treatment in the study experienced at least three bleeds a year.[2]

HAVEN 6 is a phase III, multicenter, open-label, single-arm study evaluating the safety, efficacy, pharmacokinetics and pharmacodynamics of Hemlibra in people with moderate or mild hemophilia A without factor VIII inhibitors. The primary analysis included data from 72 participants (69 men and three women) who warranted prophylaxis; 21 had mild hemophilia A without factor VIII inhibitors and 51 had moderate hemophilia A without inhibitors at a median follow-up of 55.6 weeks. At baseline, 37 participants were receiving factor VIII prophylactic treatment and 24 had target joints.[1]

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The data show that Hemlibra maintained low treated bleed rates across the study period, with 66.7% of participants experiencing no bleeds that required treatment, 81.9% experiencing no spontaneous bleeds that required treatment, and 88.9% experiencing no joint bleeds that required treatment.[1] Model-based annualized bleed rates (ABR) remained low throughout the evaluation period at 0.9 (95% CI: 0.55-1.52).

The results also show that Hemlibra's safety profile was consistent with findings across various subpopulations of people with hemophilia A, from previous HAVEN and STASEY studies, with no new safety signals observed. The most common adverse event (AE) related to treatment occurring in 10% or more people in the HAVEN 6 study was local injection site reactions (ISRs) (16.7%). Fifteen people (20.8%) reported a Hemlibra-related AE, of which the majority were local ISRs. One participant experienced a grade one thromboembolic event unrelated to Hemlibra. There were no deaths or cases of thrombotic microangiopathy, reinforcing Hemlibra's favorable safety profile.[1]

Hemlibra is approved to treat people with hemophilia A with factor VIII inhibitors in more than 110 countries worldwide and for people without factor VIII inhibitors in more than 95 countries worldwide, including the US and Japan for all severities of hemophilia A, and the EU for only severe hemophilia A. It has been studied in one of the largest clinical trial programs in people with hemophilia A with and without factor VIII inhibitors, including eight phase III studies.

About Hemlibra

Hemlibra is a bispecific monoclonal antibody created with 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[3,4]. The product was approved by the U.S. Food and Drug Administration (FDA) in November 2017, for the first time in the world, 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. In Japan, it was first approved in March 2018, and its indication was later expanded to include congenital hemophilia A without factor VIII inhibitors. Hemlibra has been approved in more than 110 countries for congenital hemophilia A with and without factor VIII inhibitors.

References

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