Innovation all for the patients

CHUGAI

Roche A member of the Roche group

New Interim Results from Phase IIIb Study Reinforce the Long-Term Safety Profile of Chugai's Hemlibra in Hemophilia A

- The second interim analysis of the STASEY study, including data from 193 people with hemophilia A, was consistent with the results from phase III HAVEN studies, with no new safety signals identified[1,2,3].
- STASEY is the largest open-label study to evaluate the safety and tolerability of Hemlibra in hemophilia A with factor VIII inhibitors.
- The data also suggest people on Hemlibra may be able to undergo certain minor surgeries without additional preventative coagulation treatment, and major surgeries could be managed with additional prophylactic coagulation factor[4].

TOKYO, July 13, 2020 -- Chugai Pharmaceutical Co., Ltd. (TOKYO: 4519) announced that the second interim analysis results from the phase IIIb STASEY study reinforce the safety profile of Hemlibra® (emicizumab) characterized in the phase III HAVEN studies[1,2,3]. In the STASEY study, Hemlibra was effective with no new safety signals identified in adults and adolescents with hemophilia A with factor VIII inhibitors, which was consistent with the previously reported safety profile[1]. The results also suggested people on Hemlibra may be able to undergo certain minor surgeries without additional preventive coagulation factor[4]. These results were presented at the International Society on Thrombosis and Hemostasis (ISTH) 2020 Virtual Congress (July 12 to 14, 2020).

"I am pleased that the large clinical study data reinforce the long-term safety and tolerability of Hemlibra for the treatment of hemophilia A with inhibitors. The long-term data will provide great clinical insights for treatment with Hemlibra," said Dr. Osamu Okuda, Chugai's President and COO. "Hemlibra is a bispecific antibody drug with a novel mechanism of action, clearly distinct from existing drugs. We will continue our efforts to collect and report clinical data so that people with hemophilia A globally can continue to use Hemlibra with confidence."

The second interim analysis of the STASEY study included data from 193 participants with hemophilia A with factor VIII inhibitors, who received Hemlibra prophylaxis once-weekly[1]. No cases of thrombotic microangiopathy (TMA) or serious thrombotic events (TEs) related to Hemlibra were reported, and no new safety signals were observed[1]. Thirty-three (17.1%) people reported a Hemlibra-related adverse event (AE)[1]. The most common AEs, occurring in 10% or more of people in the STASEY study, were common cold symptom nasopharyngitis (12.4%), headache (11.9%), and injection-site reaction (ISR) (11.4%)[1]. The ISRs reported were either mild or moderate in severity and no patients discontinued due to ISR[1]. Annualized bleed rates (ABR) were also consistent with previously reported observations from the phase III HAVEN studies[1,2,3].

CHUGAI PHARMACEUTICAL CO., LTD. Corporate Communications Dept.
1-1, Nihonbashi-Muromachi 2-chome, Chuo-ku Tokyo, 103-8324 Japan
Media Relations Group TEL:+81-(0)3-3273-0881 E-mail:pr@chugai-pharm.co.jp
Investor Relations Group TEL:+81-(0)3-3273-0554 E-mail:ir@chugai-pharm.co.jp

A separate analysis described management and outcomes of minor and unplanned major surgeries in patients receiving Hemlibra, although not a formal surgery endpoint in STASEY. Results suggest people with hemophilia A with factor VIII inhibitors who undergo certain minor surgeries whilst receiving Hemlibra may not need additional preventative coagulation factor[4]. The majority of minor surgeries (n=20/31, 64.5%) were performed without the use of preventative coagulation factor and, of these, 85% (n=17/20) did not result in treated post-operative bleeds[4]. Of the unplanned major surgeries (n=9), eight were managed with prophylactic coagulation factor, four of which resulted in bleeds managed successfully with recombinant factor VIIa[4]. These findings are consistent with results observed in a previous analysis of surgeries in the pivotal HAVEN studies[5].

STASEY is a single-arm, multicenter, open-label, phase IIIb clinical study where participants received Hemlibra for an average of 50.9 weeks. The ABR of all bleeds, including treated bleeds, treated spontaneous bleeds, treated joint bleeds and treated target joint bleeds were low, with 167 patients (85.6%) experiencing zero treated bleeds[1]. There were two TEs unrelated to Hemlibra reported. One was a ST-elevation myocardial infarction in a person with pre-existing risk factors, which the treating physician assessed as unrelated to Hemlibra[1]. The second was a hypertrophic clot at the site of a tooth extraction, a known complication of the procedure[1].

There is no participation from Japan to the STASEY study.

Sources

1. Jimenez-Yuste V et al. Second Interim Analysis Results from STASEY Trial: A Single-arm, Multicenter, Open-Label, Phase III Clinical Trial to Evaluates the Safety and Tolerability of Emicizumab Prophylaxis in People with Hemophilia A (PwHA) with FVIII inhibitors. [poster no. PB0958] International Society on Thrombosis and Haemostasis (ISTH) 2020 Congress, 12-14 July, 2020
2. Oldenburg J et al. Emicizumab Prophylaxis in Hemophilia A with Inhibitors. NEJM 2017; 377:809-818.
3. Young G et al. Emicizumab prophylaxis provides flexible and effective bleed control in children with hemophilia A with inhibitors: results from the HAVEN 2 study. Blood 2018; 132 (Supplement 1): 632.
4. Castaman G et al. Surgical Experience from the Phase III STASEY Trial of Emicizumab Prophylaxis in Persons with Hemophilia A (PwHA) with FVIII Inhibitors: Data from the Second Interim Analysis. [poster no: PB0939] International Society on Thrombosis and Haemostasis (ISTH) 2020 Congress, 12-14 July, 2020
5. Santagostino E et al. Surgical experience from four phase III studies (HAVEN 1–4) of emicizumab in persons with haemophilia A (PwHA) with or without FVIII inhibitors. [OC 60.1], Nurses and Orals Abstracts. Res Pract Thromb Haemost 2019; 3:1-228

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

For US media
Chugai Pharma USA Inc.
Casey Astringer
Tel: +1-908-516-1350
E-mail: pr@chugai-pharm.com

For European media
Chugai Pharma U.K. Ltd.
Tania Richards
Tel: +44-20-8987-5680
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

###