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Chugai Files for Additional Indication of Hemlibra for Treatment of Acquired Hemophilia A

- The application was submitted based on a phase III clinical study in Japan (AGEHA STUDY) for acquired hemophilia A
- The application is subject to priority review, based on the orphan drug designation

TOKYO, November 11, 2021 -- Chugai Pharmaceutical Co., Ltd. (TOKYO: 4519) announced that it filed an application today with the Ministry of Health, Labour and Welfare (MHLW) for approval of additional indication for the anti-coagulation factor IXa/X humanized bispecific monoclonal antibody / coagulation factor VIII substitute Hemlibra® [generic name: emicizumab (genetical recombination)] for routine prophylaxis to prevent or reduce the frequency of bleeding episodes in people with acquired blood coagulation factor VIII deficiency. In October 2021, Hemlibra received orphan drug designation from the MHLW for this setting, and the application is subject to priority review.

"Acquired hemophilia A is a disease with high unmet medical needs and designated as an intractable disease in Japan. More treatment options are needed for this disease, and I am glad that we have filed application for Hemlibra for this indication," said Dr. Osamu Okuda, Chugai's President and CEO. "Since its launch in 2018, Hemlibra has been used widely as a treatment option for congenital hemophilia A in children and adults. Chugai will continue working toward obtaining approval to contribute to the treatment of acquired hemophilia A."

This application is based on the data from the ongoing phase III clinical study in Japan (AGEHA study) for acquired hemophilia A.

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Chugai Receives Orphan Drug Designation for Hemlibra in Acquired Hemophilia A (Press release by Chugai issued on October 1, 2021)
https://www.chugai-pharm.co.jp/english/news/detail/20211001170000_851.html

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[1, 2]. 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

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in more than 100 countries for congenital hemophilia A with and without factor VIII inhibitors.

About acquired hemophilia A

Acquired hemophilia A is a disease in which inhibitors of blood coagulation factor VIII acquired, resulting in a significant decrease in factor VIII activity, leading to bleeding symptoms such as spontaneous subcutaneous bleeding and intramuscular bleeding, and serious bleeding is not rare. Acquired hemophilia A is an autoimmune disease in which autoantibodies against factor VIII are produced on the backgrounds of collagen disease, malignant tumor, and child birth[3,4]. Immunosuppressive therapy aimed at eliminating inhibitors is needed to reduce the risk of bleeding. However, since there is a risk of causing severe infections, the importance of controlling infections during the acute phase is pointed out[5]. Hemlibra is designed to demonstrate efficacy of preventing bleeding without being affected by inhibitors, and aims to change existing treatment strategies, including immunosuppressive therapies.

Sources

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2) Sampei, et al. PLoS ONE 2013; 8(2): e57479
3) Franchini M, Veneri D. Acquired coagulation inhibitor-associated bleeding disorders: an update. Hematology 2005;10:443-9.
4) Cohen AJ, Kessler CM.Acquired inhibitors.Baillieres Clin Haematol 1996;9:331-54.
5) Ichiro TANAKA, Kagehiro AMANO, Masashi TAKI, Toshiaki OKA, Michio SAKAI, Akira SHIRAHATA, et al. A 3-year consecutive survey on current status of acquired inhibitors against coagulation factors in Japan―analysis of prognostic factors―. Journal of Thrombosis and Hemostasis 2008;19:140-53.

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