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Chugai Receives Orphan Drug Designation for Hemlibra in Acquired Hemophilia A

TOKYO, October 1, 2021 -- Chugai Pharmaceutical Co., Ltd. (TOKYO: 4519) announced that its anti-coagulation factor IXa/X humanized bispecific monoclonal antibody / coagulation factor VIII substitute Hemlibra® [generic name: emicizumab (genetical recombination)], received orphan drug designation for routine prophylaxis to prevent or reduce the frequency of bleeding episodes in people with acquired blood coagulation factor VIII deficiency, from the Ministry of Health, Labour and Welfare. A Phase III clinical study with Hemlibra in this setting is currently ongoing in Japan.

“Acquired hemophilia A is a disease caused by autoantibodies (inhibitors) to factor VIII. It may cause serious bleeding, however, not all people are eligible for existing treatments due to underlying diseases, drug resistance or other various reasons, requiring more treatment options,” said Dr. Osamu Okuda, Chugai’s President and CEO. “Hemlibra is designed to reduce the risk of bleeding using Chugai’s proprietary bispecific antibody technology. Chugai will continue its efforts in clinical development to make available a drug representing a new mechanism of action for people with acquired hemophilia A as soon as possible, following its existing application in congenital hemophilia A.”

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 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.

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About orphan drugs

Based on Pharmaceuticals and Medical Devices Law, orphan drugs are designated by the Minister of Health, Labour and Welfare and granted priority review. The designation criteria are as follows: The number of patients who may use the drug is less than 50,000 in Japan; The drug is indicated for the treatment of serious diseases and there is a significant medical value such as no alternative appropriate drug or treatment, or high efficacy or safety expected compared to existing products; there is a theoretical rationale for using the product for the targeted disease and the development plan is reasonable.

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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