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Chugai Obtains Regulatory Approval for Hemlibra for Additional Indication of Acquired Hemophilia A

- The approval is based on the results of a Japanese phase III clinical study (AGEHA study) in acquired hemophilia A
- Acquired hemophilia A is one of the immune-mediated acquired coagulation factor deficiencies (designated intractable disease 288)

TOKYO, June 20, 2022 -- Chugai Pharmaceutical Co., Ltd. (TOKYO: 4519) announced that it obtained regulatory approval from the Ministry of Health, Labour and Welfare for the anti-coagulation factor IXa/X humanized bispecific monoclonal antibody/coagulation factor VIII substitute Hemlibra® [generic name: emicizumab (genetical recombination)] for an additional indication of routine prophylaxis to prevent or reduce the frequency of bleeding episodes in patients with acquired hemophilia A. Hemlibra received orphan drug designation for this indication in October 2021, and under the priority review process, the additional indication was approved seven months after the application in November 2021.

Acquired hemophilia A is one of the immune-mediated acquired coagulation factor deficiencies listed among the designated intractable diseases in Japan (designated intractable disease 288). The disease is associated with repeated episodes of sudden hemorrhage due to the development of inhibitors (autoantibodies) against blood coagulation factor VIII. Treatments widely used for the disease include immunosuppressive therapy to suppress the production of autoantibodies as well as bypassing agents (formulations that induce blood clotting responses that bypass factor VIII) to control bleeding episodes. Patients with acquired hemophilia A often experience severe bleeding, and bleeding in vital organs may potentially be life-threatening. Some patients cannot be adequately treated with existing therapies for a variety of reasons, including underlying disease and resistance to existing therapies, thus requiring expansion of treatment options.

“We are very pleased to offer Hemlibra as a treatment for acquired hemophilia A, following congenital hemophilia A. Hemlibra was created by applying Chugai's proprietary bispecific antibody technology. The approval today allows an additional measure for patients to effectively control bleeding at an earlier stage after diagnosis. We believe this will provide flexibility in the treatment strategy for acquired hemophilia A and may lead to the realization of better treatment according to each patient's condition,” said Dr. Osamu Okuda, Chugai’s President and CEO. “Chugai's mission is to dedicate ourselves to adding value for patients through innovative medicines. We will continue working with all our efforts to realize innovation for patients awaiting new treatments.”

The approval today is based on the results of the AGEHA study, a phase III clinical trial conducted in patients with acquired hemophilia A. The primary analysis of the AGEHA study will be presented on July 11 at the 30th International Society on Thrombosis and Hemostasis (ISTH, July 9-13) held online and in London, UK.

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Approval Information *Description for acquired hemophilia A

Indications:

Routine prophylaxis to prevent or reduce the frequency of bleeding episodes in patients with acquired hemophilia A

Dosage and administrations:

The usual dosage is emicizumab (genetical recombination) administered subcutaneously at 6 mg/kg (body weight) on Day 1 and 3mg/kg (body weight) on Day 2 and then subcutaneous administration of 1.5 mg/kg (body weight) once a week from Day 8.

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 are 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 basis of collagen disease, malignant tumor, and childbirth.[3,4)]

Sources

1. Kitazawa, et al. Nature Medicine 2012; 18(10): 1570
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.

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