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Chugai Files Applications for HEMLIBRA® for the Treatment of Hemophilia A without Factor VIII Inhibitors and for Extension of Dosing Interval

TOKYO, April 26, 2018 -- Chugai Pharmaceutical Co., Ltd. (TOKYO: 4519) announced today that it filed applications for its anti-coagulation factor IXa/X humanized bispecific monoclonal antibody / coagulation factor VIII substitute, "HEMLIBRA® Subcutaneous Injection 30 mg, 60 mg, 90 mg, 105 mg, and 150 mg" [generic name: emicizumab (genetical recombination)] to the Ministry of Health, Labour and Welfare (MHLW) for an additional indication of prophylactic treatment for people with hemophilia A without inhibitors to factor VIII, as well as for additional dosage and administration as a biweekly or every four-week treatment for people with hemophilia A with inhibitors to factor VIII.

These filings are based on the results from HAVEN 3 study (NCT02847637) and HAVEN 4 study (NCT03020160). HAVEN 3 study is a global phase III study evaluating HEMLIBRA subcutaneous injection, once a week and once every two weeks, in people with hemophilia A (12 years of age or older) without inhibitors to factor VIII. HAVEN 4 study is a global phase III study evaluating efficacy, safety, and pharmacokinetics of HEMLIBRA subcutaneous injection every four weeks in people with hemophilia A (12 years of age or older), with and without inhibitors to factor VIII. Based on the results from these studies, the filings were submitted for two purposes including: 1) expansion of the indication to include weekly, biweekly, or every four-week subcutaneous treatment for people with hemophilia A without inhibitors to factor VIII; and 2) addition of dosage and administration to include the biweekly or every four-week subcutaneous treatment for people with hemophilia A with inhibitors to factor VIII for whom the weekly injection has previously been approved.

"HEMLIBRA has recently been designated as a Breakthrough Therapy from the U.S. Food and Drug Administration (FDA) for the treatment of hemophilia A without inhibitors to factor VIII. We are very glad that the filing to include this indication has been successfully submitted in Japan," said Chugai's Executive Vice President, Co-Head of Project & Lifecycle Management Unit, Dr. Yasushi Ito. "The purpose of the two filings is to obtain approval for weekly, biweekly, and every four-week subcutaneous injections of HEMLIBRA regardless of the presence of inhibitors to factor VIII. Chugai is committed to seek approval so that we may deliver HEMLIBRA to people with hemophilia A without inhibitors as a new therapeutic option, as well as to further improve convenience for people with inhibitors by extending the dosing interval as soon as possible."

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

References

About the results of HAVEN 3 study

Press release issued on November 20, 2017

https://www.chugai-pharm.co.jp/english/news/detail/20171120151500.html

About the results of HAVEN 4 study

Press release issued on December 7, 2017

https://www.chugai-pharm.co.jp/english/news/detail/20171207150000.html

About the approval for HEMLIBRA for people with hemophilia A with factor VIII inhibitors in Japan

Press release issued on March 23, 2018

https://www.chugai-pharm.co.jp/english/news/detail/20180323150000.html

About Breakthrough Therapy Designation for the treatment of hemophilia A without factor VIII inhibitors in the U.S.

Press release issued on April 17, 2018

https://www.chugai-pharm.co.jp/english/news/detail/20180417140000.html