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Chugai Files a New Drug Application for Its Bispecific Monoclonal Antibody "Emicizumab" for the Treatment of Congenital Factor VIII Deficiency (Hemophilia A) with Factor VIII Inhibitors

TOKYO, July 21, 2017 -- Chugai Pharmaceutical Co., Ltd. (TOKYO: 4519) announced today that it filed a new drug application for its anti-coagulation factor IXa/X humanized bispecific monoclonal antibody emicizumab (genetic recombinant, Development Code: ACE910) to the Ministry of Health, Labour and Welfare (MHLW) for the planned indication of "Routine prophylaxis to prevent or reduce the frequency of bleeding episodes in patients with congenital factor VIII deficiency (hemophilia A) with factor VIII inhibitors."

This filing is based on the results of HAVEN 1 study (NCT02622321) and the interim analysis of HAVEN 2 study (NCT02795767), both of which have been conducted under a collaboration between Chugai, Roche and Genentech. The two studies have been carried out in hemophilia A patients with factor VIII inhibitors in order to evaluate the efficacy, safety and pharmacokinetics of the once-weekly subcutaneous injection of emicizumab, while HAVEN 1 is for adult and adolescent patients (12 years of age or older) and HAVEN 2 is for pediatric patients (younger than 12 years of age).

The data of HAVEN 1 study was published in the online version of The New England Journal of Medicine (NEJM) in July 2017.

About emicizumab/ACE910

Emicizumab is an investigational bispecific monoclonal antibody, which was developed using Chugai's proprietary antibody engineering technologies. The drug is designed to bind factors IXa and factors X, and promotes the interaction between factors IXa and factors X. In doing so, emicizumab 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)]. Emicizumab obtained an Orphan Drug Designation by the MHLW for the prevention and reduction of bleeding episodes in patients with congenital factor VIII deficiency with inhibitors in August 2016. Furthermore, the drug was designated as a Breakthrough Therapy by the US Food and Drug Administration (FDA) for the prophylactic treatment of people who are 12 years or older with hemophilia A with factor VIII inhibitor in September 2015.

1) Kitazawa, et al. Nature Medicine 2012;18(10):1570
2) Sampei, et al. PLoS ONE 2013;8:e57479

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