Kyowa Hakko Kirin Announces Initiation of Phase 3 Study in Japan of Mogamulizumab in Patients with HTLV-1 Associated Myelopathy

Tokyo, Japan, June 30th, 2017 --Kyowa Hakko Kirin Co., Ltd. (Headquarters:Tokyo; President and CEO: Nobuo Hanai, "Kyowa Hakko Kirin") announces that it has initiated a Phase 3 clinical study in Japan of mogamulizumab (code No. : KW-0761)*1 in patients with HTLV-1-associated myelopathy.

HTLV-1-associated myelopathy (HAM) manifests as inflammation in the spinal cord caused by T-lymphocytes infected with human T-cell leukemia virus type 1 (HTLV-1). The disease progresses slowly and is accompanied by symptoms such as walking difficulties, numbness, difficulty passing urine, and constipation. A 2010 epidemiological survey estimated that approximately 3,000 patients in Japan have developed HAM, where it is a designated intractable disease*2.

Mogamulizumab is a humanized monoclonal antibody targeting CC chemokine receptor 4 (CCR4). Mogamulizumab utilizes its enhanced ADCC activity to eliminate CCR4-positive T-lymphocytes infected with HTLV-1, and therefore is expected to alleviate symptoms in patients with HAM.

Kyowa Hakko Kirin started the Phase 3 study based on the results of the investigator-initiated clinical trials (Phase 1/2a study (completed) and the long-term study (ongoing)) led by Dr. Yoshihisa Yamano, Department of Rare Diseases Research, Institute of Medical Science, St. Marianna University School of Medicine. The purpose of the Phase 3 study is to evaluate the efficacy and safety of mogamulizumab in patients with HAM.

"HAM is a severe disease which causes long term myelopathic symptoms, such as lower limb paralysis, pain, dysuria, and constipation, and for which there are no currently effective treatments." said Mitsuo Satoh, Ph.D., Head of Research and Development Division of Kyowa Hakko Kirin. "We hope mogamulizumab will be a treatment option for patients with HAM."

The Kyowa Hakko Kirin Group companies strive to contribute to the health and well-being of people around the world by creating new value through the pursuit of advances in life sciences and technologies.

<Outline of the study>

Target Disease	HTLV-1-associated myelopathy (HAM)
Design	Randomized, double-blind, placebo-controlled study with an open label study
Target number of subjects	52
Primary endpoint	Improvement in the Osame's Motor Disability Score (OMDS)
Publicized information	ClinicalTrial.gov: NCT03191526

[*1] About mogamulizumab (KW-0761)

Mogamulizumab is a humanized monoclonal antibody targeting CC chemokine receptor 4 (CCR4). CCR4 is frequently expressed in certain hematologic cancer cells, but also expressed in T-lymphocytes infected with HTLV-1. Mogamulizumab is manufactured using Kyowa Hakko Kirin's POTELLIGENT® technology that is related to increased ADCC activity. It was approved for sale for the first time globally in Japan, and since March 2012 has been sold as an approved drug (brand name: POTELIGEO®) for relapsed or refractory CCR4-positive adult T-cell leukemia-lymphoma (ATL). Furthermore, mogamulizumab obtained approval for additional indications in Japan for relapsed or refractory CCR4-positive peripheral T-cell lymphoma (PTCL) and cutaneous T-cell lymphoma (CTCL) in March 2014 as well as for chemotherapy-naïve CCR4-positive ATL in December 2014.

[*2] About designated intractable diseases

Designated intractable diseases are diseases covered by medical subsidies as set forth in Article 5 of the Law for Medical Treatment of Patients with Intractable Diseases enacted in May 2014 in Japan. The designation is made by the Minister of Health, Labor, and Welfare when quality and appropriate medical treatment are judged to be imperative for patients with such a disease based on the opinion of the Health Science Council.