Innovation all for the patients

CHUGAI

Roche A member of the Roche group

Chugai receives Orphan Drug Designation for Emicizumab

- Preventing and Reducing the Frequency of Bleeding Episodes in Congenital FVIII Deficiency Patients with Inhibitors -

TOKYO, August 26, 2016 -- Chugai Pharmaceutical Co., Ltd. (TOKYO: 4519) announced that emicizumab (ACE910/RO5534262) received orphan drug designation by the Ministry of Health, Labour and Welfare for the prevention and reduction of bleeding episodes in patients with congenital FVIII deficiency (hemophilia A) who developed inhibitors to FVIII. Emicizumab is a humanized bispecific antibody for subcutaneous injection under development for hemophilia A.

Hemophilia A is an inherited disorder in which the blood coagulation reaction does not proceed normally due to a congenital lack or impaired function of coagulation FVIII. People with hemophilia A begin to experience severe bleeding repeatedly in various body parts such as joints and muscle in their childhood. Above all, frequent joint bleeding may cause hemophilic arthropathy, which affects daily functions and impairs quality of life. It is estimated that there are approximately 5,000 patients with hemophilia A in Japan*. Patients with severe hemophilia A currently receive regular FVIII replacement therapy to reduce the frequency of bleeding. For those patients who develop antibodies (inhibitors) against FVIII, bypassing agents are used to promote blood-clotting reaction independently from FVIII.

“With the results from Japanese Phase I/II study (ACE002JP) of emicizumab we have seen so far, I have high hopes that the drug can bring innovation to current treatment for hemophilia A both in terms of efficacy and improvement in quality of life,” said Chugai’s Senior Vice President, Head of Project & Lifecycle Management Unit, Dr. Yasushi Ito. “Particularly, there are high unmet medical needs for patients with inhibitors. We are committed to deliver emicizumab to patients as early as possible to contribute for the realization of better treatments.”

Primary patient enrolment has been completed in the global Phase III study in adolescents/adults with hemophilia A who acquired FVIII inhibitor, and the study is now under the follow-up phase.

About orphan drug designation

Orphan drug designation is granted in conjunction with priority review status by the Minister of Health, Labour and Welfare according to the Pharmaceuticals and Medical Devices Law. The designation criteria requires the eligible number of patients to be less than 50,000 in Japan, there are no other replaceable drugs or treatment available, significant medical value such as high efficacy or safety can be expected compared to existing products, a theoretical rationale for using the product for the targeted disease, and a feasible development plan.

*Project entrusted by Ministry of Health, Labour and Welfare. Nationwide Survey on Coagulation Disorders 2015. Published by Japan Foundation for AIDS Prevention.

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