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Chugai's Emicizumab Showed Continued Benefits in Patients with Hemophilia A
—Update from Japanese P I/II study presented at WFH 2016—

TOKYO, July 28, 2016 -- Chugai Pharmaceutical Co., Ltd. (TOKYO: 4519) announced today that latest data from an ongoing Japanese Phase I/II study (ACE002JP) of emicizumab was presented at the World Federation of Hemophilia 2016 World Congress in Orlando, Florida, United States. Emicizumab is a bispecific antibody for subcutaneous injection under development for hemophilia A.

ACE002JP is an extension study of the patient part of a Phase I study (ACE001JP), which was conducted to investigate safety and exploratory prophylactic efficacy profiles of emicizumab in Japanese hemophilia A patients both with and without FVIII inhibitors. The latest data analysis continued to show a promising profile of once-weekly subcutaneous injection of emicizumab in terms of safety and prophylactic efficacy, regardless of the presence of factor VIII inhibitors. The mean follow-up period were 32.6, 27.0 and 21.4 months in the 0.3, 1 and 3 mg/kg groups respectively.

"This new long-term follow-up data supports emicizumab's continued prophylactic efficacy in hemophilia A patients both with and without inhibitors," said Chugai's Senior Vice President, Head of Project & Lifecycle Management Unit, Dr. Yasushi Ito. "We anticipate that emicizumab will bring innovation to the current treatment for hemophilia A and deliver significant value to patients and their families."

Emicizumab was designated as a Breakthrough Therapy by the US Food and Drug Administration in September 2015. Currently, two Phase III global studies in adolescents/adults and children with hemophilia A who acquired FVIII inhibitors are ongoing respectively in collaboration with Roche, Chugai's strategic alliance partner. A Phase III global study in patients without FVIII inhibitors is also planned to start later this year. Furthermore, a Phase III global study investigating administration once every four weeks is also planned.

The data of the patient part of ACE001JP was published in The New England Journal of Medicine in May 2016.
http://www.nejm.org/doi/full/10.1056/NEJMoa1511769

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[Outline of the study]

Cohort	Number of patients		Dose
	Patients with inhibitors	Patients without inhibitors	
C-1	4	2	1*, 0.3** mg/kg
C-2	4 i)	2	3*, 1** mg/kg
C-3	3	3 ii)	3 mg/kg

*Initial dose, **Second and subsequent doses

i) One patient discontinued emicizumab due to injection site erythema of mild intensity during Phase I study

ii) One patient did not transit to extension study since prior treatment was sufficiently efficacious.

[Study results]

SAFETY

- 18 patients all experienced adverse events. Those adverse events were of mild or moderate intensity, except for 2 severe cases (appendicitis and mesenteric hematoma).
- No thromboembolic adverse events or clinically significant abnormality of coagulation tests were observed.
- 7 of 18 patients experienced local injection site reactions which were manageable.
- No neutralizing ADA (anti-drug antibodies) were observed.

EFFICACY

- ABR (Annualized Bleeding Rate) remained low and 8 of 18 patients had experienced zero bleeds.
- Breakthrough bleeds were successfully treated with standard episodic treatment, FVIII products or bypassing agents.
- The median ABR and AJBR (Annualized Joint Bleeding Rate) at pre- and post-administration of emicizumab in each cohort are as follows:

Cohort	Median ABR (times)		Median AJBR (times)		Median [range] follow-up (months)
	Pre-emicizumab	Post-emicizumab	Pre-emicizumab	Post-emicizumab	
C-1 (N=6)	32.5	1.4	27.4	1.1	32.6 [32.2-33.3]
C-2 (N=6)	18.3	0.2	15.2	0.2	27.0 [8.2-28.5]
C-3 (N=6)	15.2	0.0	9.1	0.0	21.4 [11.1-22.5]

About Chugai

Chugai Pharmaceutical is one of Japan's leading research-based pharmaceutical companies with strengths in biotechnology products. Chugai, based in Tokyo, specializes in prescription pharmaceuticals and is listed on the 1st section of the Tokyo Stock Exchange. As an important member of the Roche Group, Chugai is actively involved in R&D activities in Japan and abroad. Specifically, Chugai is working to develop innovative products which may satisfy the unmet medical needs, mainly focusing on the oncology area.

In Japan, Chugai's research facilities in Gotemba and Kamakura are collaborating to develop new pharmaceuticals and laboratories in Ukima are conducting research for technology development for industrial production. Overseas, Chugai Pharmabody Research based in Singapore is engaged in research focusing on the generation of novel antibody drugs by utilizing Chugai's proprietary innovative antibody engineering technologies. Chugai Pharma USA and Chugai Pharma Europe are engaged in clinical development activities in the United States and Europe.

The consolidated revenue in 2015 of Chugai totaled 498.8 billion yen and the operating income was 90.7 billion yen (IFRS Core basis).

Additional information is available on the internet at http://www.chugai-pharm.co.jp/english.

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