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# Chugai receives Orphan Drug Designation for Tocilizumab in Systemic Scleroderma

TOKYO, March 17, 2016 - Chugai Pharmaceutical Co., Ltd. (TOKYO: 4519) announced today that it received from the Minister of Health, Labour and Welfare, a notification of orphan drug designation for human anti-human IL-6 receptor monoclonal antibody "tocilizumab," a drug under development for treatment of systemic scleroderma.

"Systemic scleroderma is a refractory chronic disease with limited therapeutic options and high unmet medical needs. Thus healthcare providers and patients have been waiting a long time for a new drug," said Chugai's Director and Executive Vice President, Dr. Yutaka Tanaka. "In order to be of some help for them, we are committed to develop and obtain early approval of tocilizumab."

Systemic scleroderma is designated as an intractable disease, by the Ministry of Health, Labour and Welfare. It manifests skin hardening, and internal organs are affected associated with its progression. Its etiology is yet to be fully elucidated. Current therapeutic options are only symptomatic in nature, such as corticosteroids and immunosuppressants and no established treatment is in place that can alleviate signs and symptoms as a whole. A new therapeutic agent with improved efficacy has been long awaited.

Chugai has been engaged in clinical development of tocilizumab as a drug for systemic scleroderma, in cooperation with its strategic alliance partner, Roche. A Phase III placebo-controlled clinical trial "focuSSced" is now under way. In February 2015, the results of the previous study, a Phase II clinical trial "faSScinate" indicated the possibility of improved efficacy of tocilizumab compared to existing drugs. Based on this finding, tocilizumab has been designated as a "Breakthrough Therapy" by the U.S. Food and Drug Administration.

**About orphan drugs**

Based on Pharmaceuticals and Medical Devices Law, orphan drugs are designated by the Minister of Health, Labour and Welfare, and granted priority review. The designation criteria includes the following: The number of patients who may use the drug is less than 50,000 in Japan; there is no alternative appropriate drug or treatment; there is a significant medical value such as high efficacy or safety expected compared to existing products; there is a theoretical rationale for using the product for the targeted disease and the development plan is reasonable.

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