April 25, 2011

Center for iPS Cell Research and Application, Kyoto University Dainippon Sumitomo Pharma Co., Ltd

## <u>CiRA and DSP Research the Creation of a New Treatment for Rare Intractable</u> <u>Diseases</u>

The Center for iPS Cell Research and Application (CiRA), Kyoto University (Location: Kyoto; Director: Shinya Yamanaka) and Dainippon Sumitomo Pharma Co., Ltd. (DSP; Headquarters: Osaka, Japan; President: Masayo Tada) announce the signing of an agreement for 5 years of joint research with the aim to create treatment for a rare intractable disease.

This joint research collaboration between industry and academia, focusing on a rare intractable disease caused by genetic mutation, aims to use induced pluripotent stem cells (iPS cells) to identify the pathognomonic mechanism of the disease, identify the most disease-specific target molecule from various pathological changes of signal transduction, and screen specific inhibitors against the signal pathway. As a result, the aim is to create an epoch-making treatment to suppress detrimental conditions in patients.

(Reference)

Joint Research Outline

Research Period: March 2011 – March 2016 (5 Years)

Research Objective and Shared Roles:

(CiRA and DSP)

To use induced pluripotent stem cells (iPS cells) to identify the pathognomonic mechanism of the disease, identify the most disease-specific target molecule from various pathological changes of signal transduction, and screen specific inhibitors against the signal pathway.

(DSP)

To synthesize, evaluate and optimize candidate compounds, and develop the treatment for the disease found through the joint research .

## Target disease:

This joint research project focuses on a certain disease designated as a rare intractable disease by the Ministry of Health, Labour and Welfare, Japan. At the present time, there is no effective treatment against this disease, because due to the nature of the disease traditional approaches are inapplicable to define its pathognomonic mechanism. However, it is expected that iPS cell technology will make an innovative breakthrough in the discovery of treatment of the disease. Due to the sensitivity of the research, the specific name of the rare disease is not disclosed.

About the Center for iPS Cell Research and Application (CiRA), Kyoto University

Established on April 1, 2010, CiRA is a world-first core institute dedicated to leading iPS cell research. CiRA has a system to promote the seamless translation of fundamental research to preclinical and clinical applications. Location: 53 Kawahara-cho, Shogoin, Sakyo-ku, Kyoto 606-8507, Japan. Director: Shinya Yamanaka, M.D., Ph.D. URL: http://www.cira.kyoto-u.ac.jp/e/

About Dainippon Sumitomo Pharma Co., Ltd.

Dainippon Sumitomo Pharma supplies REPLAGAL<sup>®</sup>, the therapeutic agent for Anderson-Fabry disease, and is progressing the development of WT1 cancer peptide vaccine "WT4869" aiming for treatment of Myelodysplastic syndrome (MDS). DSP works on the continued development of treatment for rare diseases.

Location: Osaka, Japan.

President: Masayo Tada

URL: http://www.ds-pharma.co.jp

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