



February 26, 2018

Sumitomo Dainippon Pharma Co., Ltd. JCR Pharmaceuticals Co., Ltd.

# Sumitomo Dainippon Pharma and JCR Sign a License Agreement on Blood-Brain Barrier Penetration Technology for Creation of a Therapeutic Agent for Central Nervous System Diseases

Sumitomo Dainippon Pharma Co., Ltd. (Head Office: Osaka, Osaka; President and CEO: Masayo Tada) ("Sumitomo Dainippon Pharma") and JCR Pharmaceuticals Co., Ltd. (Headquarters: Ashiya, Hyogo; Chairman and President: Shin Ashida) ("JCR") announced today that, on February 26, 2018, the two companies have signed a license agreement on rights possessed by JCR concerning a substance created by applying JCR's J-Brain Cargo<sup>®</sup> blood-brain barrier (BBB) penetration technology to a candidate compound of Sumitomo Dainippon Pharma's choice (hereinafter referred to as the "Substance"), and whose rights are shared by both companies, following an agreement on development of the Substance as a therapeutic agent for specified central nervous system diseases by Sumitomo Dainippon Pharma.

Under the license agreement with JCR, Sumitomo Dainippon Pharma retains the exclusive rights to research, develop, and market the Substance indicated for specified central nervous system diseases in Japan and North America, in return for which Sumitomo Dainippon Pharma will provide up to  $\pm 3.7$  billion in total to JCR including an upfront payment and development milestone payments at each development phase. Following the commercial launch of the Substance, Sumitomo Dainippon Pharma will also pay royalties in accordance with the sales amount and milestone payments on sales to JCR that will be calculated on the basis of sales target achievements. Sumitomo Dainippon Pharma has also option rights to additional indications and territory (China).

Sumitomo Dainippon Pharma expects that the Substance will become an innovative therapeutic agent in the central nervous system area, which has high unmet medical needs.

JCR has conducted Phase I and Phase II clinical studies of JR-141, a J-Brain Cargo<sup>®</sup>-applied enzyme preparation for treatment of Hunter syndrome, and has obtained positive results. Aside from those in-house programs on J-Brain Cargo<sup>®</sup> platform, this is JCR's first license agreement on a candidate drug using J-Brain Cargo<sup>®</sup>.

(Reference information)

[Background of the license agreement] As announced in the press release dated June 17, 2015, the two companies concluded a feasibility study agreement to begin studying the potential of the Substance as a therapeutic agent for specified central nervous system diseases. Now that the Substance has met the mutually established criteria, a license agreement has been signed.

#### [About J-Brain Cargo<sup>®</sup>]

J-Brain Cargo enables delivery of target substances across the BBB through transferrin receptor expressed on the surface of vascular endothelial cells. JCR's nonclinical study confirmed BBB penetration of J-Brain Cargo<sup>®</sup> -applied target substance as 20 to 100 times the target substance alone. It is an innovative technology which will provide pharmaceutical compounds of low to high molecules with the capacity to pass through the BBB. A substantially large portion of the pharmaceutical injected intravenously should be able to reach inside the brain and show its efficacy. Improvements are expected for psychiatric or neurological disorders for which little improvements have been possible.

### [About Sumitomo Dainippon Pharma]

Sumitomo Dainippon Pharma's goal is to create innovative pharmaceutical products. Psychiatry & Neurology as well as Oncology represent our focus therapeutic areas containing significant unmet medical needs. It is channeling its R&D energies also into two new fields: diseases where no approved drugs exist as well as regenerative medicine/cell therapy.

### [About JCR Pharmaceuticals]

JCR is engaged in the creation of innovative, first-in-class drugs with a primary focus on rare diseases including lysosomal storage diseases. As a specialty pharma, we strive to contribute to better health for people and medicine of tomorrow through our research, development, manufacture and sales of pharmaceutical products generated from our proprietary biotechnologies and technologies for cell therapy and regenerative medicine.

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