



February 12, 2019
JCR Pharmaceuticals Co., Ltd.

Translation

JCR Announces Presentation on JR-171 for Hurler Syndrome at the 15th Annual *WORLDSymposium*[™] 2019

JCR Pharmaceuticals Co., Ltd. (TSE: 4552; Chairman and President Shin Ashida; "JCR") announced that it has given a poster presentation on the non-clinical evaluation of JR-171. JR-171 is a blood-brain barrier (BBB)-penetrating acid alpha-L iduronidase product candidate for the treatment of patients with Hurler syndrome, to which J-Brain Cargo[®], JCR's proprietary BBB technology, is applied. The poster presentation was held at the 15th Annual *WORLDSymposium*[™] 2019* in Orlando, Florida (February 4-8, 2019).

The following is a summary of the presentation.

Hurler syndrome (mucopolysaccharidosis type I) is a lysosomal storage disorder caused by an inherited defect or mutation of the lysosomal enzyme alpha-L iduronidase (IDUA) gene, resulting in a decrease or loss of the IDUA enzymatic activity and an accumulation of the substrates, heparan sulfate (HS) and dermatan sulfate (DS), in cells throughout the body. In conventional enzyme replacement therapy, the enzyme is unable to cross the BBB, hence its efficacy on the central nervous system (CNS) symptoms being limited. JCR has thereupon produced JR-171, a BBB-penetrating IDUA using J-Brain Cargo[®], as a part of its efforts to develop therapeutic enzymes capable of effectively treating the CNS symptoms.

JR-171, as well as IDUA, was intravenously administered to monkeys in order to compare the distributions of both substances in the brain. Strong signals from JR-171 were detected in the brain in comparison with IDUA, indicating the penetration of JR-171 into the brain.

JR-171 was also administered to the mice models for Hurler syndrome to evaluate its effect on the reduction in the substrates. Significant reduction in the HS and DS concentrations was noted in the brain as well as in the peripheral tissues when compared with the mice administered with the existing therapeutic enzyme.

These results demonstrate that, in non-clinical evaluation, JR-171 distributes into the brain by crossing the BBB, and that JR-171 has the potential to exert beneficial therapeutic effects on the CNS symptoms in Hurler syndrome.

* *WORLDSymposium*[™] is an international symposium held annually in the United States with a focus on basic research to clinical application in the lysosomal diseases. <http://www.worldsymposia.org/>

[About JCR Pharmaceuticals]

JCR is a specialty pharma engaged in the research, development, manufacture and marketing of biopharmaceuticals and regenerative medicine with a focus on rare diseases. Its philosophy, "Contributing towards people's healthcare through pharmaceutical products" drives JCR to create innovative pharmaceutical products as value-added treatment options for the under-served patient community.

[Cautionary Statement Regarding Forward-Looking Statements]

This document contains forward-looking statements that are subject to known and unknown risks and uncertainties, many of which are outside our control. Forward-looking statements often contain words such as “believe,” “estimate,” “anticipate,” “intend,” “plan,” “will,” “would,” “target” and similar references to future periods. All forward-looking statements regarding our plans, outlook, strategy and future business, financial performance and financial condition are based on judgments derived from the information available to us at this time. Factors or events that could cause our actual results to be materially different from those expressed in our forward-looking statements include, but not limited to, a deterioration of economic conditions, a change in the legal or governmental system, a delay in launching a new product, impact on competitors’ pricing and product strategies, a decline in marketing capabilities relating to our products, manufacturing difficulties or delays, an infringement of our intellectual property rights, an adverse court decision in a significant lawsuit and regulatory actions.

This document involves information on pharmaceutical products (including those under development). However, it is not intended for advertising or providing medical advice. Furthermore, it is intended to provide information on our company and businesses and not to solicit investment in securities we issue.

Except as required by law, we assume no obligation to update these forward-looking statements publicly or to update the factors that could cause actual results to differ materially, even if new information becomes available in the future.

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