News Release

December 13, 2017
JCR Pharmaceuticals Co., Ltd.

Translation

JCR Announces Positive Topline Results from Phase 1/2 Clinical Trial of JR-141 in Hunter Syndrome

JCR Pharmaceuticals Co., Ltd. (TSE 4552: Chairman and President Shin Ashida, "JCR") today announced topline results from its Phase 1/2 clinical trial for its JR-141, a BBB-penetrating recombinant iduronate-2-sulfatase product, in patients with Hunter syndrome. JR-141 is the first of its kind to apply JCR's proprietary blood-brain-barrier (BBB) technology, J-Brain Cargo®.

This Phase 1/2 open-label, dose-escalating study was designed to evaluate the safety, exploratory efficacy and pharmacokinetics of intravenous administration of JR-141 in Hunter patients. There were no safety concerns. After a 4-week intravenous administration of JR-141 given once weekly, heparan sulfate (HS) in cerebrospinal fluid as the biomarker for central nervous system (CNS) disorders, significantly dropped by an average of approximately 30 percent in all patients. The magnitude of HS reduction was comparable with that seen in the animal study results obtained to date, suggesting the medicinal effect of JR-141 crossing into the BBB and acting on the CNS. In view of such, the treatment with JR-141 is expected to contribute to improve CNS disorders which have not been addressed by the existing enzyme replacement therapy.

The results from Phase 1/2 clinical trial of JR-141, along with its preclinical study results, will be announced at the 14th Annual WORLD*Symposium*™ 2018*. Based on these results, JCR plans to initiate a Phase 2/3 program to further evaluate the safety and efficacy of JR-141 as a potential treatment for Hunter syndrome.

As we progress with the development of JR-141, JR-162, a J-Brain Cargo®-applied acid alpha-glucosidase for treating Pompe disease, and JR-171, a J-Brain Cargo®-applied alpha-L iduronidase for treating Hurler syndrome as announced this October, we will harness J-Brain Cargo® technology as the platform for developing a robust pipeline of innovative ERT products for other lysosomal storage disorders with CNS involvement. Ongoingly, JCR, as a specialty pharma, will proactively engage in the research and development of treatment options for the patients with rare diseases.

This clinical trial result is expected to have a minor impact on consolidated financial results for the year ending on March 31, 2018.

* WORLD*Symposium*™ 2018, is a symposium held annually in the U.S. which is designed to cover from the basic research to clinical application of discoveries related to lysosomal storage disorders.

[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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