News Release

June 1, 2018
JCR Pharmaceuticals Co., Ltd.

Translation

JCR Announces Completion of PMDA Review of Phase 3 Clinical Trial of JR-141 for Hunter Syndrome

JCR Pharmaceuticals Co., Ltd. (TSE 4552: Chairman and President Shin Ashida, "JCR") announced today that Pharmaceutical and Medical Devices Agency (PMDA), completed the review of the Phase 3 clinical trial plan for JR-141. JR-141 is a blood-brain-barrier (BBB)-penetrating recombinant iduronate-2-sulfatase product candidate for the treatment of patients with Hunter syndrome to which J-Brain Cargo®, JCR's proprietary BBB technology, is applied. JCR anticipates initiating the study in August 2018.

This Phase 3 clinical trial in Japan is designed to evaluate the effect of JR-141 on changes on in systemic and central nervous system (CNS) symptoms over a 12 month period in 20 patients with Hunter syndrome. The primary endpoint is a biomarker in cerebrospinal fluid (CSF).

As already announced, the Phase 1/2 clinical trials conducted in Japan last year demonstrated the safety of JR-141. Results from a study using a biomarker showed as its effect on the improvement of CNS symptoms, in addition to its efficacy on systemic symptoms comparable to idursulfase. Based on those results, JR-141 was designated under the SAKIGAKE Designation System in Japan in March 2018.

Ongoingly, completion of this Phase 3 clinical trial as planned will enable JCR to submit its first application for marketing approval of JR-141 in Japan in 2019, ahead of other countries. Working towards global development, JCR anticipates initiating the Phase 2 clinical trial in Brazil this month.

Following JR-141, we will harness J-Brain Cargo® technology as the platform for developing a robust pipeline of innovative enzyme replacement therapy products for other lysosomal storage disorders with CNS involvement. JCR, as a specialty pharma, will continue to proactively engage in the research and development of treatment options for the patients with rare diseases.

This clinical trial is expected to have a minor impact on consolidated financial results for the year ending on March 31, 2019.

[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.

Investors & Media:
JCR Pharmaceuticals Co., Ltd.
Corporate Communications
ir-info@jcrpharm.co.jp

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