News Release

July 31, 2020
JCR Pharmaceuticals Co., Ltd.

Translation

JCR Announces Completion of the Phase 1/2 Clinical Trial Notification of JR-171 for mucopolysaccharidosis type I (MPS I)
-Phase 1/2 clinical trial is estimated to start in Japan in September 2020-

JCR Pharmaceuticals Co., Ltd. (TSE 4552; Chairman and President: Shin Ashida; "JCR") announced today the successful completion of the review for the phase 1/2 clinical trial notification of JR-171 for MPS I (Hurler syndrome, Hurler-Scheie syndrome, Scheie syndrome) by the Pharmaceuticals and Medical Devices Agency (PMDA). JR-171 is a blood-brain-barrier (BBB)-penetrating recombinant α-L-iduronidase, the product candidate for the treatment of patients with MPS I, to which J-Brain Cargo®, JCR's proprietary BBB penetration technology, is applied.
This study is planned to be conducted in Japan, Brazil, and the United States as the JCR's first global clinical trial. We aim to start this study in September of this year in Japan prior to other countries.

MPS I, like MPS II (Hunter syndrome) is an autosomal recessive disease and a type of lysosomal storage disorder (LSD). It is characterized by a deficiency of a specific enzyme called, α-L-iduronidase, responsible for the degradation of mucopolysaccharides. Symptoms include central nervous system (CNS) symptoms, joint lesions, short stature, corneal clouding, heart valve disease, and hepatosplenomegaly. An enzyme replacement therapy exists for the treatment of systemic signs and symptoms, but due to its inability to cross the BBB, it leaves the CNS symptoms unaddressed.

In this study, MPS I patients will be treated with JR-171 for 12 weeks to evaluate the safety as the primary endpoint and exploratory efficacy and pharmacokinetics as secondary endpoints. The summary of this study is also registered on clinicaltrials.gov.

In addition to JR-171, JCR has developed a series of therapeutic enzymes for LSDs that harness J-Brain Cargo® technology. As a specialty pharma in the rare disease arena, JCR will accelerate its research and development activities to contribute to the treatment for a broader patient population.

This new drug development is expected to have a minor impact on JCR's consolidated financial results for the year ending March 31, 2021.

[About JCR Pharmaceuticals]
JCR is a specialty pharma company engaged in the research, development, manufacturing 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 populations.

[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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