



October 30, 2020  
JCR Pharmaceuticals Co., Ltd.

Translation

### **JCR Announces First Patient Dosed in Phase 1/2 Global Clinical Trial of JR-171 for mucopolysaccharidosis type I (MPS I)**

JCR Pharmaceuticals Co., Ltd. (TSE 4552; Chairman and President: Shin Ashida; “JCR”) announced today that the dosing of the first patient in Phase 1/2 global clinical trial of JR-171 was initiated on October 28, 2020 (JST). JR-171 is a blood-brain-barrier (BBB)-penetrating recombinant  $\alpha$ -L-iduronidase, the product candidate for the treatment of patients with MPS I (Hurler syndrome, Hurler-Scheie syndrome, Scheie syndrome), to which J-Brain Cargo®, JCR’s proprietary BBB penetration technology, is applied.

MPS I is a lysosomal storage disorder (LSD) characterized by multiple somatic and central nervous system (CNS) signs and symptoms. An enzyme replacement therapy exists for the treatment of somatic 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 at most 12 weeks to evaluate the safety of JR-171 as the primary endpoint and exploratory efficacy and pharmacokinetics as secondary endpoints. This study is planned to be conducted in Japan, Brazil, and the United States as the JCR’s first global clinical trial. In Brazil, JR-171 has obtained Rare Disease Designation and the start of this study has been approved. In the US, JCR is preparing for the start of this study as well.

The summary of this study is also registered on [clinicaltrials.gov](https://clinicaltrials.gov) (Identifier : [NCT04227600](https://clinicaltrials.gov/ct2/show/study/NCT04227600))

In addition to JR-171, JCR has developed a series of therapeutic enzymes that harness J-Brain Cargo® technology for –the treatment of LSDs with CNS sequelae. As a specialty pharma in the rare disease arena, JCR will accelerate its research and development activities in the LSD space to make this innovation available to a broader patient population outside of clinical trials.

The clinical trial plan for JR-171 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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