



August 9, 2018  
JCR Pharmaceuticals Co., Ltd.

Translation

### **JCR Initiates Phase 3 Clinical Trial of JR-141 for Hunter Syndrome**

JCR Pharmaceuticals Co., Ltd. (TSE 4552; Chairman and President: Shin Ashida; “JCR”) announced today the initiation of a Phase 3 clinical trial of JR-141 in Japan. 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<sup>®</sup>, JCR’s proprietary BBB technology, is applied.

This trial is designed to evaluate the effect of JR-141 on changes in the systemic and central nervous system (CNS) symptoms over a 12-month period in 20 patients with Hunter syndrome. Primary endpoint is changes of biomarkers in the cerebrospinal fluid. The early results from the study will be shared as soon as they become available

JR-141 has been designated under the SAKIGAKE Designation System in Japan in March 2018. JCR anticipates submitting application for the marketing approval of JR-141 in Japan in 2019. Working towards global development of JR-141, JCR has initiated a Phase 2 clinical trial in Brazil in June 2018.

Following JR-141, we harness J-Brain Cargo<sup>®</sup> technology as a platform to develop a robust pipeline of innovative enzyme replacement therapy products for other lysosomal storage disorders as well. JCR, as a pharma dedicated to speciality care, will continue to proactively engage in 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.*

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