News Release

March 30, 2021

JCR Pharmaceuticals Co., Ltd.

Translation

EMA grants Orphan Drug Designation to JR-171
for the Treatment of Mucopolysaccaridosis Type I (MPS I)

Mar. 30, 2021 -- JCR Pharmaceuticals Co., Ltd. (TSE 4552; Chairman and President: Shin Ashida; "JCR") announced today that European Medicines Agency (EMA) has granted orphan drug designation to JR-171, an investigational drug for the treatment of mucoplysaccharidosis type I (MPS I, or Hurler, Hurler-Scheie and Scheie syndrome). JR-171 is a blood-brain-barrier (BBB)-penetrating form recombinant α-L-iduronidase that was developed using JCR's proprietary J-Brain Cargo® BBB technology.

JR-171 received the orphan drug designation from the US Food Drug Administration (FDA) in February 2021. Currently JR-171 is undergoing a global Phase 1/2 clinical trial and the first patient in Japan was dosed in October, 2020 and in Brazil in March, 2021. The trial is also scheduled for enrolling patients in the US. The summary of this study is registered on clinicaltrials.gov.

JCR, as a specialty pharma in the rare disease arena, will continue to proactively engage in research and development of transformative treatment options for patients with rare diseases. Therefore, following JR-171, JCR plans to harness its J-Brain Cargo® technology platform and progress its robust pipeline of innovative enzyme replacement therapy (ERT) products for additional lysosomal storage disorders (LSDs).

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

Orphan designation (EMA)

The EMA implements orphan designation for promoting new drug development for rare diseases in which the prevalence of the condition in the EU must not be more than 5 in 10,000. Designated drugs are granted market exclusivity for ten years in the EU, as well as scientific guidance.

About MPS I (Hurler, Hurler-Scheie, Scheie syndrome)

MPS I is an autosomal recessive LSD caused by a deficiency of α-L-iduronidase, an enzyme that breaks down glycosaminoglycans (mucopolysaccharides) in the body. The number of patients

with MPS I worldwide is estimated to be approximately 3,600 (according to JCR research). MPS I gives rise to a wide range of somatic and neurological symptoms. A major limitation to current ERT is that it does not address central nervous system (CNS) symptoms because of the enzyme's inability cross the BBB.

About J-Brain Cargo® Technology

JCR's first-in-class proprietary technology, J-Brain Cargo®, enables the development of therapies that cross the BBB and penetrate the CNS. The CNS complications of LSDs are often severe, resulting in developmental delays, an impact on cognition and, above all, poor prognosis, which affect patients' independence as well as the quality of life of patients and their caregivers. With J-Brain Cargo®, JCR seeks to address the unresolved clinical challenges of LSDs by delivering the enzyme to both the body and the brain.

About JCR Pharmaceuticals Co., Ltd.

JCR Pharmaceuticals Co., Ltd. (TSE 4552) is a global specialty pharmaceuticals company that is redefining expectations and expanding possibilities for people with rare and genetic diseases worldwide. We continue to build upon our 45-year legacy in Japan while expanding our global footprint into the US, Europe, and Latin America. We improve patients' lives by applying our scientific expertise and unique technologies to research, develop, and deliver next-generation therapies. Our approved products in Japan include therapies for the treatment of growth disorder, Fabry disease, acute graft-versus host disease, and renal anemia. Our investigational products in development worldwide are aimed at treating rare diseases including MPS I (Hurler, Hurler-Scheie and Scheie syndrome), MPS II (Hunter syndrome), Pompe disease, and more. JCR strives to expand the possibilities for patients while accelerating medical advancement at a global level. Our core values – reliability, confidence, and persistence – benefit all our stakeholders, including employees, partners, and patients. Together we soar. For more information, please visit https://www.jcrpharm.co.jp/en/site/en/.

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 are 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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