News Release

February 12, 2021

JCR Pharmaceuticals Co., Ltd.

Translation

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

Feb. 12, 2021 -- JCR Pharmaceuticals Co., Ltd. (TSE 4552; Chairman and President: Shin Ashida; "JCR") announced today that the US Food and Drug Administration (FDA) has granted orphan drug designation to JR-171, an investigational drug for the treatment of MPS I (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 is a recombinant fusion protein of an antibody against the human transferrin receptor and α-L-iduronidase, the enzyme that is missing or malfunctioning in subjects with MPS I. By crossing the BBB through transferrin receptor-mediated transcytosis it is expected to be effective against central nervous system (CNS) symptoms of the disease, thereby addressing a significant unmet need in the treatment of MPS I. Uptake into somatic cells is mediated through the mannose-6-phosphate receptor.
JR-171 is currently undergoing a global Phase 1/2 clinical trial and the first patient was dosed in October, 2020 in Japan. The trial is also scheduled for enrolling patients in Brazil and the United States.

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). 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.

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

Orphan drug designation

The US FDA implements orphan drug designation for promoting new drug development for rare diseases affecting up to 200,000 patients in the United States. Designated drugs are granted market exclusivity for seven years in the United States, as well as support in the form of

government subsidies and tax deductions on clinical R&D expenses.

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 in worldwide is estimated at 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 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 lysosomal storage diseases (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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