News Release

January 21, 2022
JCR Pharmaceuticals Co., Ltd.

Translation

EC grants Orphan Drug Designation to JR-441
for the Treatment of Mucopolysaccharidosis Type III A (MPS IIIA)

Jan. 21, 2022 -- JCR Pharmaceuticals Co., Ltd. (TSE 4552; Chairman and President: Shin Ashida; "JCR") announced today that European Commission (EC) has granted orphan drug designation to JR-441, an investigational drug for the treatment of mucopolysaccharidosis type III A (MPS IIIA, or Sanfilippo type A). JR-441 is a blood-brain barrier (BBB)-penetrating form of recombinant heparan N-sulfatase that was developed using JCR's proprietary J-Brain Cargo® BBB-penetrating technology.

With the Orphan Drug Designation, JR-441 will be eligible for various incentives to encourage the development in the European Union (EU).

MPS IIIA is a lysosomal storage disorder (LSD) characterized by multiple somatic and severe central nervous system (CNS) signs and symptoms. Type III A is relatively severe.
JR-441 is a recombinant fusion protein of antibody against the human transferrin receptor and heparan N-sulfatase, the enzyme missing or malfunctioning in subjects with MPS IIIA. By crossing the BBB, JR-441 is expected to be effective also against CNS symptoms of the disease, thereby addressing a significant unmet need in the treatment of MPS IIIA. Currently, JCR is preparing to start a global clinical trial for JR-441 in the first half of 2023. Lining up withJR-141 (approved in Japan for the treatment of MPS II) and JR-171 (for the treatment of MPS I), JR-441 will be the third asset in JCR's LSD portfolio entering clinical development stage.

Following JR-441, JCR plans to harness its J-Brain Cargo technology platform and progress its robust pipeline of innovative enzyme replacement therapies (ERTs) for other 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.

There is no impact on our consolidated business results for this fiscal year ending on March 31, 2022 related to the matter.

Orphan designation (EU)

The EC implements orphan designation for promoting new drug development for rare diseases in which the prevalence of the condition in the EU affecting no more than five in 10,000 people. Designated drugs are granted market exclusivity for ten years in the EU, as well as scientific guidance. Fee reductions are also available depending on the status of the sponsor and the type of service required.

About Sanfilippo syndrome (MPS III)

Sanfilippo syndrome is an autosomal recessive disease caused by a deficiency of the enzymes that metabolize mucopolysaccharides within the body. The disease is classified into four subtypes (A, B, C, and D) according to the respective deficient enzymes. Symptoms include accumulation of heparan sulfate in tissues throughout the body. Notably, the rapidly progressive form of the disease frequently affects neurocognitive development, peaking at 2 or 3 years of age, before subsequent
deterioration leading to a complete loss of speech by the age of 7 or 8. Progression further gives rise to symptoms such as sleep disorders, hepatosplenomegaly, and seizures.

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 46-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, MPS II (Hunter syndrome), 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), 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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