News Release

October 5, 2021
JCR Pharmaceuticals Co., Ltd.

Translation

US FDA grants Fast Track Designation for JR-171
for the Treatment of Mucopolysaccaridosis Type I (MPSI)

Oct. 5, 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 Fast Track designation for the investigational drug JR-171 for the treatment of Mucopolysaccaridosis (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.

MPS I is a lysosomal storage disorder (LSD) characterized by multiple somatic and central nervous system (CNS) signs and symptoms.
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.

JR-171 currently started a global Phase 1/2 clinical trial part 2 that is conducted in Japan, US and Brazil. The summary of this study is also registered on clinicaltrials.gov (Identifier : NCT04453085).
Granting Fast Track designation for JR-171 allows JCR to interact more frequently with the FDA. The Agency may also consider reviewing portions of the marketing application before the sponsor submits the complete application if FDA determines, after preliminary evaluation of clinical data that the fast-track product may be effective.
In addition, JR-171 was designated as an orphan drug by the FDA in February and by European Commission in March of this year.

Following JR-171, JCR plans to harness its J-Brain Cargo technology platform and progress its robust pipeline of innovative ERT products for other 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.

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

FDA First Track Designation

The FDA Fast track is a process designed to facilitate the development, and expedite the review of drugs to treat serious conditions and fill an unmet medical need.The purpose is to enable early delivery of important new drugs to the patients.A drug that receives Fast Track designation may be allowed more frequent meetings with FDA to discuss the drug's development plan, followed bypriority reviewandan accelerated approvalwhen relevant criteria are met.

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

MPS I is an autosomalrecessive LSD caused by a deficiency ofα-L-iduronidase, an enzyme that breaks down glycosaminoglycans (mucopolysaccharides) in the body. The number of patients with MPS Iin worldwide is estimated at approximately 3,600(according to JCR research). MPS Igives 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 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, 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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