



July 11, 2023
JCR Pharmaceuticals Co., Ltd.

Translation

JCR Receives Approval to Start Global Phase I/II Clinical Trial for JR-441 for the Treatment of Mucopolysaccharidosis Type III A (MPS IIIA)

July 11, 2023-- JCR Pharmaceuticals Co., Ltd. (TSE 4552; Chairman and President: Shin Ashida; "JCR") announced today that the PEI (Paul-Ehrlich Institute) accepted the clinical trial application (CTA) for JR-441, a blood-brain barrier (BBB) penetrating form of heparan N-sulfatase that was developed using JCR's proprietary J-Brain Cargo® BBB-penetrating technology. 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 Mucopolysaccharidosis Type III A (MPS IIIA; Sanfilippo Syndrome Type A).

MPS IIIA is a lysosomal storage disorder (LSD) characterized by multiple somatic and neurological signs and symptoms with no established standard of care. While MPS III is a heterogeneous disease, type IIIA is typically characterized by an earlier onset than other forms of MPS III. While less prevalent in Japan, MPS IIIA is considered the most prevalent form of Sanfilippo Syndrome globally. Preclinical evidence indicated that JR-441 may address the sequelae of MPS IIIA in individuals affected with the disease.

The CTA for a phase I/II clinical trial submitted in Germany has been approved by the PEI and JCR is finalizing the protocol and other requirements with the aim to start recruiting in the third quarter 2023.

JCR, a pharmaceutical company having developed the proprietary BBB-penetrating technology J-Brain Cargo®, is focusing on the development of treatments for LSDs with CNS signs and symptoms. JR-441 is the third program advancing into global clinical stage, following JR-141 for treatment of MPS II (Hunter syndrome) and JR-171 for treatment of MPS I (Hurler syndrome). Furthermore, JCR intends to start clinical trials on four new programs from its LSD pipeline by 2028.

There is no impact on our consolidated business results for this fiscal year ending on March 31, 2024 related to the matter.

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, which peaks around 2 or 3 years of age, before subsequently deteriorating, resulting in a complete loss of speech by the age of 7 or 8. Progression further gives rise to symptoms such as sleep disorders, hepatosplenomegaly, seizures, and neurobehavioral abnormalities.

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 48-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), MPS III A and B (Sanfilippo syndrome type A and B), 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.

Contact:

Investors & Media:

JCR Pharmaceuticals Co., Ltd.

Corporate Communications

ir-info@jp.jcrpharm.com

###