News Release

August 29, 2024
JCR Pharmaceuticals Co., Ltd.

JCR Pharmaceuticals Announces Completion of Clinical Trial Notification Process in Japan for Phase I Study of JR-441 for Mucopolysaccharidosis Type IIIA

Hyogo, Japan – Aug. 29, 2024 -- JCR Pharmaceuticals Co., Ltd. (TSE 4552; Chairman and President: Shin Ashida; "JCR") announced today that the completion of the regulatory review by the Pharmaceuticals and Medical Devices Agency (PMDA) for the clinical trial notification for the Phase I study of JR-441 in individuals with mucopolysaccharidosis type IIIA (MPS IIIA; Sanfilippo syndrome type A).

JR-441 is a recombinant form of heparan N-sulfatase that is developed using JCR's proprietary J-Brain Cargo® blood-brain barrier (BBB)-penetrating technology. JR-441 addresses a significant unmet medical need by crossing the BBB and targeting the neurological symptoms of MPS IIIA within the central nervous system.

"MPS IIIA is a rare and life-threatening lysosomal storage disorder for which there are currently no approved treatment options for individuals living with this devastating disease, and there is a dire need to address the neurological signs and symptoms of MPS IIIA," said Shin Ashida, Chairman and President of JCR Pharmaceuticals. "We are pleased that the PMDA's review of the Phase I study plan is complete, and that clinical development program is progressing. We are committed to continuing to advance the JR-441 clinical development program with the goal of meeting the unmet medical needs of this patient community. We look forward to sharing updates on the program when they are available."

The European Commission (EC) and the U.S. Food and Drug Administration (FDA) granted JR-441 orphan drug designation (ODD) for the treatment of MPS IIIA. JR-441 is currently being studied in an ongoing Phase I/II clinical trial in Germany (JR-441-101, NCT06095388), apart from this Japanese Phase I study.

The impact of this announcement on JCR's consolidated financial results for this fiscal year (ending March 2025) is expected to be minor.

About Mucopolysaccharidosis Type IIIA (Sanfilippo Syndrome Type A)

Mucopolysaccharidosis type IIIA, or Sanfilippo syndrome type A, is an autosomal recessive disease caused by pathogenic mutations in the *SGSH* gene, encoding a lysosomal enzyme involved in the degradation of heparan sulfate. The accumulation of heparan sulfate in several types of cells of the body, especially in the central nervous system in the brain, results in severe neurological deterioration, cognitive impairment, mild somatic involvement, and shortened lifespan.

About JCR Pharmaceuticals Co., Ltd.

JCR Pharmaceuticals Co., Ltd. (TSE 4552) is a global specialty pharmaceuticals company that is expanding possibilities for people with rare and genetic diseases worldwide. We continue to build upon our 49-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, MPS II (Hunter syndrome), 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, MPS IIIA 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. 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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