News Release

October 31, 2024
JCR Pharmaceuticals Co., Ltd.

JCR Pharmaceuticals Initiates First Dosing in Phase I Clinical Trial of JR-441 for Mucopolysaccharidosis Type IIIA

Hyogo, Japan – October 31, 2024 – JCR Pharmaceuticals Co., Ltd. (TSE 4552; "JCR") announced the initiation of the first patient dosing in Japan in the Phase I clinical trial of JR-441, an investigational enzyme replacement therapy for the treatment of mucopolysaccharidosis type IIIA (MPS IIIA, also known as Sanfilippo Syndrome Type A). JR-441 is a proprietary recombinant heparan N-sulfatase capable of crossing the blood-brain barrier (BBB).

MPS IIIA is a rare genetic disorder characterized by severe central nervous system (CNS) symptoms, for which there is currently no approved treatment. Preclinical studies have demonstrated the potential of JR-441 to address CNS-related symptoms associated with MPS IIIA.

This open-label, multicenter, single-arm study aims to assess the safety profile, biological effect, and pharmacokinetic profile of JR-441 in patients aged 1 to under 18 years with MPS IIIA.

"MPS IIIA has been one of the most significant challenges in the treatment of CNS symptoms," said Dr. Kimitoshi Nakamura, Professor of Pediatrics at the Graduate School of Medical Sciences, Kumamoto University, and the Medical Expert of the study. "This novel therapeutic approach represents a new era for managing the condition. We have been eagerly awaiting the opportunity to offer this treatment, and I am hopeful that its effectiveness will be confirmed in the clinical setting, ultimately improving the daily lives of both patients and their families."

JR-441 received Orphan Drug Designation from both the European Commission (EC) and the U.S. Food and Drug Administration (FDA), with a Phase I/II trial underway in Germany, which began in 2023 (JR-441-101, NCT06095388).

For more details on the Japanese Phase I trial, visit the Clinical Research Submission and Disclosure System (JR-441-JP11, jRCT2071240053).

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.

Contact:

Investors & Media:

JCR Pharmaceuticals Co., Ltd.

Corporate Communications

ir-info@jp.jcrpharm.com

###