News Release

December 25, 2024
JCR Pharmaceuticals Co., Ltd.

JCR Pharmaceuticals Receives MHLW Orphan Drug Designation for JR-441 for the Treatment of Mucopolysaccharidosis Type IIIA (MPS IIIA)

Hyogo, Japan – December 25, 2024 – JCR Pharmaceuticals Co., Ltd. (TSE 4552; "JCR") announced today that the Ministry of Health, Labour and Welfare (MHLW) of Japan granted orphan drug designation (ODD) to JR-441, an investigational drug for the treatment of mucopolysaccharidosis type IIIA (MPS IIIA, or Sanfilippo syndrome type A). JR-441 is a blood brain barrier (BBB)-penetrating form of recombinant heparan N-sulfatase developed using JCR's proprietary J-Brain Cargo® BBB-penetrating technology.

JR-441 received ODDs from the European Commission (EC) in January 2022 and from the U.S. Food and Drug Administration (FDA) in December 2023.
JR-441 is an investigational drug currently being studied in two clinical trials: a Phase I/II study in Germany (JR-441-101, NCT06095388) and a Phase I study in Japan (JR-441-JP11, jRCT2071240053).

About Orphan Drug Designation (ODD)

Under the orphan drug designation system, MHLW takes special measures to support and promote research activities for the development of drugs for rare diseases. Drugs satisfying the following criteria may be designated as orphan drugs: They are targeting a condition with an incidence of fewer than 50,000 patients in Japan or their intended use is for the treatment of a designated intractable disease. The drugs should be indicated for the treatment of serious diseases including difficult-to-treat diseases for which there are high unmet medical needs, such as there is no approved medicinal product or higher efficacy or safety is expected compared with an approved medicinal product. Moreover, the applicant for ODD needs to have enough resources and plans for development of its product as an orphan drug in Japan.

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.
MPS IIIA is a designated intractable disease as "lysosomal disease" in Japan, for which there is currently no approved treatment.

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 belief – benefit all our stakeholders, including patients, partners, and employees. 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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