News Release

December 14, 2023
JCR Pharmaceuticals Co., Ltd.

Translation

U.S. FDA Grants Orphan Drug Designation to JR-441 for the Treatment of Mucopolysaccharidosis Type IIIA (MPS IIIA)

December 14, 2023-- JCR Pharmaceuticals Co., Ltd. (TSE 4552; Chairman and President: Shin Ashida; "JCR") announced that the U.S. Food and Drug Administration ("FDA") 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 that was developed using JCR's proprietary J-Brain Cargo® BBB-penetrating technology.

JR-441 previously received ODD by the European Commission (EC) in January 2022. JCR is currently conducting a global Phase I/II clinical trial for JR-441, with the first patient with MPS IIIA dosed in October 2023. The summary of this clinical trial can be found on ClinicalTrials.gov under the NCT identifier NCT06095388.

"We are pleased that the FDA granted Orphan Drug Designation to JR-441, underscoring the importance of advancing new treatments for patients living with this devastating and life-threatening disease," said Shin Ashida, Chairman and President of JCR. "There are currently no treatment options for patients with MPS IIIA. We look forward to advancing JR-441 in clinical development and bringing this very important treatment to patients with MPS IIIA as quickly and safely as possible."

With the ODD, JR-441 will be eligible for various incentives to encourage the development in the U.S.

In addition, JCR has a robust development pipeline of enzyme replacement therapies (ERTs) based on its J-Brain Cargo® technology platform. JCR will continue to proactively engage in research and development of transformative treatment options for patients in need.

There is minor impact on our consolidated business results for this fiscal year ending on March 31, 2024 related to the matter.

Orphan Drug Designation in the U.S.

The U.S. FDA's Office of Orphan Products Development grants orphan status to drugs being developed to treat, prevent, or diagnose a rare disease or condition affecting fewer than 200,000 people in the U.S. The designation provides significant incentives to promote the development of the drug including the potential for market exclusivity for seven years upon FDA approval, eligibility for tax credits for qualified clinical trials, and waiver of Prescription Drug User Fee Act Application fee.

About MPS III (Sanfilippo syndrome)

Sanfilippo syndrome is an autosomal recessive disease caused by a deficiency of the respective 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 result in accumulation of heparan sulfate in tissues throughout the body. Notably, the rapidly progressive form of the disease frequently affects neurocognitive development, peaking at 2 or 3 years of age, before subsequent deterioration leading to a complete loss of speech usually by the age of 7 or 8. Progression further gives rise to symptoms such as sleep disorders, hepatosplenomegaly, seizures and many other signs and symptoms.

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.

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