News Release

February 10, 2021

JCR Pharmaceuticals Co., Ltd.

Translation

FDA grants Fast Track Designation for JR-141 for the treatment of Mucopolysaccharidosis Type II (Hunter Syndrome)

Feb. 10, 2021 -- JCR Pharmaceuticals Co., Ltd. (TSE 4552; Chairman and President: Shin Ashida; "JCR") announced today that the US Food and Drug Administration (FDA) has granted Fast Track designation for the investigational drug JR-141 (INN: pabinafusp alfa) for the treatment of Mucopolysaccharidosis (MPS) II (Hunter syndrome).
JR-141 is a blood-brain-barrier (BBB)-penetrating recombinant iduronate-2-sulfatase product candidate for the treatment of patients with Hunter syndrome, to which J-Brain Cargo®, JCR's proprietary BBB technology, is applied.

Hunter syndrome is an X-linked recessive Lysosomal storage disorders caused by a deficiency of iduronate-2-sulfatase, an enzyme that breaks down glycosaminoglycans (mucopolysaccharides) in the body. Hunter syndrome gives rise to a wide range of somatic and central nervous system (CNS) symptoms. A major limitation to the current enzyme replacement therapy (ERT) is that it does not address CNS symptoms because of the enzyme's inability cross the BBB.

JR-141 is a recombinant fusion protein of antibody against the human transferrin receptor and idursulfase, the enzyme missing or malfunctioning in subjects with MPS II. It is expected to be effective against central nervous system (CNS) symptoms by crossing the BBB through transferrin receptor-mediated transcytosis using J-Brain Cargo®, JCR's proprietary BBB technology. It thereby has the potential to fulfill a serious unmet need for the treatment of MPS II. Currently, JCR is preparing to start a global Phase 3 clinical trial for JR-141 in the US, Brazil and Europe. The FDA recently cleared the JR-141 IND application and informed JCR that the study may proceed.,
Granting Fast Track designation for JR-141 allows JCR to interact more frequently with the FDA. The Agency may also consider reviewing portions of the marketing application before the sponsor submits the complete application if FDA determines, after preliminary evaluation of clinical data that the fast track product may be effective.

Following JR-141, JCR plans to harness its J-Brain Cargo® technology platform and progress its robust pipeline of innovative ERT products for other lysosomal storage disorders (LSDs). JCR, as a specialty pharma in the rare disease arena, will continue to proactively engage in research and development of transformative treatment options for patients with rare diseases.

This designation is expected to have a minor impact on JCR's consolidated financial results for the year ending on March 31, 2021.

FDA First Track Designation

The FDA Fast track is a process designed to facilitate the development, and expedite the review of drugs to treat serious conditions and fill an unmet medical need. The purpose is to enable early delivery of important new drugs to the patients. A drug that receives Fast Track designation may be allowed more frequent meetings with FDA to discuss the drug's development plan, followed by priority review and an accelerated approval when relevant criteria are met.

About JR-141

JR-141 is a recombinant fusion protein of an antibody against the human transferrin receptor and idursulfase, the enzyme that is missing or malfunctioning in subjects with Hunter syndrome. It incorporates J-Brain Cargo®, JCR's proprietary BBB-penetrating technology, to cross the BBB through transferrin receptor-mediated transcytosis, and its uptake into cells is mediated through the mannose-6-phosphate receptor. This novel mechanism of action is expected to make JR-141 effective against the CNS symptoms of Hunter syndrome.

In pre-clinical trials, JCR has confirmed both high-affinity binding of JR-141 to transferrin receptors, and passage across the BBB into neuronal cells, as evidenced by electron microscopy. In addition, JCR has confirmed enzyme uptake in various brain tissues. The company has also confirmed a decrease in substrate accumulation in an animal model of Hunter syndrome.[1,2]

In several clinical trials of JR-141, JCR obtained evidence of reduced HS concentrations in the CSF, a biomarker for assessing effectiveness against CNS symptoms; these results were consistent with those obtained in pre-clinical studies. Clinical studies have also demonstrated positive effects of JR-141 on CNS symptoms.[3]

JR-141 has already been filed and under review for regulatory approval in Japan and Brazil.

About J-Brain Cargo® Technology

JCR's first-in-class proprietary technology, J-Brain Cargo®, enables the development of therapies that cross the BBB and penetrate the CNS. The CNS complications of diseases are often severe, resulting in developmental delays, an impact on cognition and, above all, poor prognosis, which affect patients' independence as well as the quality of life of patients and their caregivers. With J-Brain Cargo®, JCR seeks to address the unresolved clinical challenges of LSDs by delivering the enzyme to both the body and the brain.

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 45-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), Pompe disease, 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.

References

[1] Sonoda H, Morimoto H, Yoden E, et al. A blood-brain-barrier-penetrating anti-human transferrin receptor antibody fusion protein for neuronopathic mucopolysaccharidosis II. Molecular Therapy. 2018;26(5):1366-1374. (DOI: https://doi.org/10.1016/j.ymthe.2018.02.032)

[2] Morimoto H, Kida S, Yoden E, et al. Clearance of heparan sulfate in the brain prevents eurodegeneration and neurocognitive impairment in MPS II mice. Molecular Therapy. 2021 Jan 25;S1525-0016(21)0027-7. DOI: https://doi.org/10.1016/j.ymthe.2021.01.027)

[3] Okuyama T, Eto Y, Sakai N, et al. Iduronate-2-sulfatase with anti-human transferrin receptor antibody for neuropathic mucopolysaccharidosis II: a phase 1/2 trial. Molecular Therapy. 2019;27(2):456-464. (DOI: https://doi.org/10.1016/j.ymthe.2018.12.005)

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