News Release

March 29, 2021
JCR Pharmaceuticals Co., Ltd.

Translation

JR-141 (Pabinafusp Alfa) for Hunter Syndrome
Notice on the Publication of the Results of the Phase 2 Clinical Trial in Brazil in Molecular Therapy

Mar. 29 -- JCR Pharmaceuticals Co., Ltd. (TSE 4552; Chairman and President: Shin Ashida; "JCR") announced today that the results of the phase 2 clinical trial in Brazil of JR-141 (pabinafusp alfa) for the treatment of mucopolysaccharidosis II (MPS II; Hunter syndrome) have been published in the electronic edition of Molecular Therapy, the official journal of American Society of Gene and Cell Therapy. JR-141 is a blood-brain-barrier (BBB)-penetrating recombinant iduronate-2-sulfatase product for the treatment of patients with MPS II, to which J-Brain Cargo®, JCR's proprietary BBB technology, is applied.
JCR has received Marketing Approval for pabinafusp alfa in Japan in March 2021, while, in December 2020, it has filed an application to the Brazilian Health Surveillance Agency (Agência Nacional de Vigilância Sanitária [ANVISA]) for marketing authorization of JR-141 for the treatment of patients with MPS II. JCR is also preparing to start a global Phase 3 clinical trial for JR-141 in the US, Brazil and Europe. The FDA recently accepted the IND application for JR-141 to start the planned clinical study. (ClinicalTrials.gov Identifier: NCT04573023).

A summary of the article is as follows.

◆ Title:
Iduronate-2-sulfatase fused with anti-human transferrin receptor antibody, pabinafusp alfa, for treatment of neuronopathic and non-neuronopathic mucopolysaccharidosis II: Report of a phase 2 trial in Brazil

◆ Digital Object Identifier: https://doi.org/10.1016/j.ymthe.2021.03.019

◆ Summary
A 26-week, open-label, randomized, parallel-group phase 2 study was conducted in Brazil to evaluate the safety and efficacy of intravenously administered pabinafusp alfa at 1.0, 2.0, and 4.0 mg/kg/week in 20 MPS-II patients. The safety profiles in the three dosage groups were similar. Neurodevelopmental evaluation suggested positive neurocognitive signals. The 2.0 mg/kg group, which demonstrated marked reductions in substrate concentrations in the CSF, serum, and urine, was considered to provide the best dose regarding safety and efficacy of the drug candidate.

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.

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