News Release

July 29, 2021
JCR Pharmaceuticals Co., Ltd.

Translation

JR-141 (Pabinafusp Alfa) for Hunter Syndrome
Notice on the Publication of a case report in Japan in JIMD Reports

Jul. 29 -- JCR Pharmaceuticals Co., Ltd. (TSE 4552; Chairman and President: Shin Ashida; "JCR") announced today that a case report based on a subject in the phase 3 clinical trial for JR-141 in Japan (INN: pabinafusp alfa) for the treatment of mucopolysaccharidosis II (MPS II; Hunter syndrome) have been published in JIMD reports, the official journal of the Society for the Study of Inborn Errors of Metabolism. JR-141 is a blood-brain-barrier (BBB)-penetrating recombinant iduronate-2-sulfatase product for the treatment of patients with MPS II, which applies JCR's proprietary J-Brain Cargo®,BBB technology.
The report about two siblings with the same genetic variants highlights the importance of early treatment of MPS II with a BBB-penetrating form of idursulfase.
In May 2021 JCR has started to market JR-141 as "IZCARGO® I.V. infusion 10mg" in Japan . In December 2020, an application for marketing authorization n was filed with the Brazilian Health Surveillance Agency (Agência Nacional de Vigilância Sanitária [ANVISA]) for the treatment of patients with MPS II. JCR is also preparing to initiate a global phase 3 clinical trial for JR-141 which will enroll patients at sites in the US, Brazil and Europe. The US FDA recently accepted the IND application for JR-141 (ClinicalTrials.gov Identifier: NCT04573023).

A summary of the article is as follows.

◆ Title:
Divergent developmental trajectories in two siblings with neuropathic mucopolysaccharidosis type II (Hunter syndrome) receiving conventional and novel enzyme replacement therapies: a case report

◆ Digital Object Identifier:
https://doi.org/10.1002/jmd2.12239

◆ Summary
Two cases of MPS-II in Japanese siblings are reported who shared the same G140V mutation in the IDS gene but showed markedly contrasting developmental trajectories following ERT(enzyme replacement therapy). Sibling 1 was diagnosed at 2 years of age, started undergoing conventional ERT shortly afterwards, and scored a developmental quotient (DQ) of 53 on the Kyoto Scale of Psychological Development at 4 years of age. Sibling 2 was diagnosed prenatally, received conventional ERT from the age of 1 month through 1 year and 11 months, when he switched to pabinafusp alfa. He attained a DQ of 104 at age 3 years and 11 months, along with significant declines in heparan sulfate concentrations in the cerebrospinal fluid. This marked difference in neurocognitive development highlights the importance of early initiation of ERT with a BBB-penetrating enzyme in patients with neuropathic MPS-II.

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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