News Release

October 15, 2020
JCR Pharmaceuticals Co., Ltd.

Translation

JR-141 (Pabinafusp Alfa) for Hunter Syndrome
Notice on the Publication of the Results of the Phase 2/3 Clinical Trials in Molecular Therapy

JCR Pharmaceuticals Co., Ltd. (TSE 4552; Chairman and President: Shin Ashida; "JCR") announced today that the results of the phase 2/3 clinical trials 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. In September, JCR has filed an application with the Ministry of Health, Labour and Welfare of Japan (MHLW) for marketing approval of JR-141 under the SAKIGAKE Designation System in Japan, while another application for marketing approval in Brazil is being prepared. A global phase 3 clinical trial for the treatment of neuronopathic and attenutated patients with MPS II will be initiated soon (ClinicalTrials.gov Identifier: NCT04573023).

A summary of the article is as follows.

◆ Title:
A Phase 2/3 Trial of Pabinafusp Alfa, IDS Fused with Anti-Human Transferrin Receptor Antibody, Targeting Neurodegeneration in MPS-II

◆ Digital Object Identifier: https://doi.org/10.1016/j.ymthe.2020.09.039

◆ Summary

Pabinafusp alfa ("JR-141") is a novel enzyme drug that crosses the BBB by transcytosis via transferrin receptors. In order to establish its efficacy and safety, a multicenter, single-arm, open-label phase 2/3 clinical trial was conducted in 28 Japanese patients with mucopolysaccharidosis II (MPS-II, Hunter syndrome) by intravenous administrations of 2.0 mg/kg of pabinafusp alfa for 52 weeks.
The primary efficacy endpoint was changes in heparan sulfate (HS) concentrations in the cerebrospinal fluid (CSF). Secondary endpoints included assessments of neurocognitive development for central efficacy, and changes in plasma HS and dermatan sulfate (DS) concentrations for peripheral efficacy. HS concentrations in the CSF significantly decreased from baseline to week 52 ($p<0.001$), suggesting continuous inhibition of substrate accumulations in the CNS, i.e. hitherto unaddressed progressive neurodegeneration. Evaluations of neurocognitive developments showed positive changes in 21 of the 28 patients. Serum HS and DS concentrations, liver and spleen volumes, and other assessments

suggested the peripheral efficacy of pabinafusp alfa comparable to that of idursulfase. Drug-related adverse events were mild or moderate in severity, transient, and manageable. The results establish delivery across the BBB of pabinafusp alfa as an effective therapeutic for treating both the CNS and peripheral symptoms of patients with MPS-II.

[About JCR Pharmaceuticals]

JCR is a specialty pharma company engaged in the research, development, manufacturing and marketing of biopharmaceuticals and regenerative medicine with a focus on rare diseases. Its philosophy, “Contributing towards people’s healthcare through pharmaceutical products” drives JCR to create innovative pharmaceutical products as value-added treatment options for the under-served patient populations.

[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 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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