October 19, 2018
JCR Pharmaceuticals Co., Ltd.

Translation

Notice of Orphan Drug Designation by the US Food Drug Administration for JR-141 for Hunter Syndrome

JCR Pharmaceuticals Co., Ltd. (TSE 4552; Chairman and President: Shin Ashida; "JCR") announced today the orphan drug designation of JR-141 for Hunter Syndrome being granted by the US Food Drug Administration (FDA). 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.

In March 2018, JR-141 was designated under the SAKIGAKE Designation System in Japan by the Ministry of Health, Labour and Welfare. Currently, the drug is undergoing Phase 3 clinical trials and JCR plans to submit an application for marketing approval in 2020. Furthermore, as an initial step in the global development of JR-141, JCR initiated a Phase 2 clinical trial in Brazil in June 2018. Having received the orphan drug designation from the FDA, JCR is now moving ahead with discussion of specific development plans in the United States and other areas.

Following JR-141, JCR is harnessing J-Brain Cargo® technology as a platform to develop a robust pipeline of innovative enzyme replacement therapy products for other lysosomal storage disorders as well. JCR, as a pharma dedicated to specialty care, will continue to proactively engage in research and development of 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, 2019.

Orphan drug designation

The US FDA implements orphan drug designation for promoting new drug development for rare diseases affecting up to 200,000 patients in the United States. Designated drugs are granted market exclusivity for seven years in the United States, as well as support in the form of government subsidies and tax deductions on clinical R&D expenses.

[About JCR Pharmaceuticals]

JCR is a specialty pharma engaged in the research, development, manufacture 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 community.

[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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