



February 28, 2019
JCR Pharmaceuticals Co., Ltd.

Translation

JCR Receives EMA Orphan Designation for JR-141 in Hunter Syndrome

JCR Pharmaceuticals Co., Ltd. (TSE 4552; Chairman and President: Shin Ashida; "JCR") announced today the orphan designation of JR-141 for Hunter Syndrome being granted by European Medicines Agency (EMA). 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.

JR-141 was designated under the SAKIGAKE Designation System in Japan by the Ministry of Health, Labour and Welfare in March 2018, and received the orphan drug designation from the US Food Drug Administration in October 2018. Currently, the drug is undergoing a Phase 3 clinical trial so that an application for its marketing approval can be filed in 2020. As an initial step in the global development of JR-141, JCR initiated a Phase 2 clinical trial in Brazil in June 2018, whilst development strategies for other areas are being planned as well.

Following JR-141, JCR harnesses its J-Brain Cargo[®] technology platform to develop a robust pipeline of innovative enzyme replacement therapy products for other lysosomal storage disorders. JCR, as a pharma dedicated to specialty care, will continue to proactively engage in research and development of treatment options for the 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 designation (EMA)

The EMA implements orphan designation for promoting new drug development for rare diseases in which the prevalence of the condition in the EU must not be more than 5 in 10,000. Designated drugs are granted market exclusivity for ten years in the EU, as well as scientific guidelines.

[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.

Investors & Media:

JCR Pharmaceuticals Co., Ltd.

Corporate Communications

ir-info@jcrpharm.co.jp

END

###