



February 3, 2020  
JCR Pharmaceuticals Co., Ltd.

Translation

**JCR to Present at the 16th Annual WORLDSymposium™ 2020**

JCR Pharmaceuticals Co., Ltd. (TSE 4552; Chairman and President: Shin Ashida; “JCR”) announced today that it has given an oral presentation and three posters at the 16th Annual WORLDSymposium™ 2020\*1 in Orlando, Florida, USA (February 10-13, 2020). These presentations highlight JCR’s development pipeline for lysosomal storage disorders; JR-141, a blood-brain barrier (BBB)-penetrating iduronate-2-sulfatase for Hunter syndrome, and JR-162, J-Brain Cargo®\*2-applied acid α-glucosidase for Pompe disease.

**The details of these presentations will be announced after the conference.**

**1. An oral presentation**

We will report 26-week results from a phase 3 study in Japan of JR-141. JR-141 is a BBB-penetrating recombinant iduronate-2-sulfatase product candidate for the treatment of patients with Hunter syndrome, to which J-Brain Cargo®, a JCR’s proprietary BBB technology, is applied.

Title	Presented on
Therapy for mucopolysaccharidosis II with an intravenous blood-brain barrier-crossing enzyme (JR-141): 26-week results from a phase 3 study in Japan suggesting significant efficacy against central nervous system and systemic symptoms	February 12 <sup>th</sup> 16:00-16:15 EST

**2. Poster presentations**

The 26-week results from a phase 3 study of JR-141 in Japan as well as the results from a phase 2 study thereof in Brazil will be reported. Also, the results from a non-clinical study of JR-162 will be reported. JR-162 is a BBB-penetrating acid α-glucosidase (GAA) product candidate for the treatment of patients with Pompe disease, to which J-Brain Cargo® is applied.

Title	Presented on
Results from a phase 2 trial of a blood-brain barrier penetrating enzyme (JR-141) in patients with MPS II in Brazil	February 10 <sup>th</sup> 16:30-18:30 EST
Therapy for mucopolysaccharidosis II with an intravenous blood-brain barrier-crossing enzyme (JR-141): 26-week results from a phase 3 study in Japan suggesting significant efficacy against central nervous system and systemic symptoms	February 11 <sup>th</sup> 16:30-18:30 EST

A novel approach to CNS dysfunction of Pompe disease with a fusion protein consisting of anti-transferrin receptor antibody and GAA enzyme	February 12 <sup>th</sup> 16:30-18:30 EST
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**\*1 WORLDSymposium™**

An international symposium held annually in the United States with a focus on the basic research to clinical application in the lysosomal diseases.

<http://www.worldsymposia.org/>

**\*2 J-Brain Cargo®**

J-Brain Cargo® is the innovative drug delivery system utilizing anti-transferrin receptor antibodies. In JR-141, being developed as a product candidate for the treatment of patients with Hunter syndrome, we expect improvement of not only systemic symptoms but also central nervous system (CNS) symptoms with J-Brain Cargo®, which allows penetrating BBB. In JR-162, being developed as a product candidate for the treatment of patients with Pompe disease, which mainly shows impairment of muscle tissues, we expect not only direct therapeutic effects on muscle tissues via transferrin receptor but also, by penetrating BBB, on nerve cells that control the muscles.

**[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 under-served patient communities.

**[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](mailto:ir-info@jcrpharm.co.jp)

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