News Release

February 4, 2021

JCR Pharmaceuticals Co., Ltd.

Translation

JCR Pharmaceuticals to Present at the 17th Annual WORLD*Symposium*™ 2021

Feb. 4, 2021 -- JCR Pharmaceuticals Co., Ltd. (TSE 4552; Chairman and President: Shin Ashida; "JCR") announced today that it will present nine posters at the virtual 17th Annual WORLD*symposium*™2021, to be held February 8-12, 2021(EST). These presentations demonstrate the potential benefits of the investigational therapies in JCR's development pipeline – and of J-Brain Cargo®, JCR's proprietary technology that delivers medicine across the blood-brain barrier – for the treatment of lysosomal storage disorders. Many of JCR's presentations will highlight JR-141 (pabinafusp alfa), a recombinant iduronate-2-sulfatase product candidate for the treatment of patients with mucopolysaccharidosis type II (MPS II, or Hunter syndrome).

Development code: JR-141 (pabinafusp alfa), BBB-penetrating iduronate-2-sulfatase (rDNA origin)

Indication: Mucopolysaccharidosis type II (Hunter syndrome)

Title	[Poster No]. Presented on
Reduction of heparan sulfate in the brain by pabinafusp alfa results in prevention of neurodegeneration and neurocognitive impairment in a mouse model of mucopolysaccharidosis II	[163] February 8th 14:30-15:30 EST
Exploration of the efficacy of pabinafusp alfa (JR-141) on neurocognitive development in Hunter syndrome (MPS-II): 52-week data from clinical trials in Japan and Brazil	[84] February 10th 14:30-15:30 EST
Drug delivery across the blood-brain barrier and resultant heparan sulfate in the cerebrospinal fluid in patients with Hunter syndrome (MPS-II): an integrated analysis of 25-week Japanese and Brazilian data on pabinafusp alfa (JR-141)	[176] February 10th 14:30-15:30 EST
Therapy for mucopolysaccharidosis type II with an intravenous blood-brain barrier-crossing enzyme (JR-141): Phase III global clinical trial design	[242] February 11th 14:30-15:30 EST
A comparison of developmental trajectories in sibling cases with neuropathic MPS-II receiving conventional and novel enzyme replacement therapies	[LB-50] February 12th 14:30-15:30 EST

Development code: JR-171, BBB-penetrating α-L-iduronidase (rDNA origin)

Indication: Mucopolysaccharidosis type I (Hurler, Hurler-scheie and Scheie syndrome)

Title	[Poster No.] Presented on
Phase I/II clinical trial design for a novel therapy for mucopolysaccharidosis type I with an intravenously administered blood-brain barrier-crossing enzyme	[99] February 11th 14:30-15:30 EST

Development code: JR-162, J-Brain Cargo®–applied acid α-glucosidase (rDNA origin)

Indication: Pompe disease

Title	[Poster No.] Presented on
Usefulness of hexose tetrasaccharide as a biomarker for monitoring glycogen accumulation in peripheral tissues and brain in Pompe disease	[94] February 11th 14:30-15:30 EST

Development code: JR-441, BBB-penetrating heparan N-sulfatase (rDNA origin)

Indication: Mucopolysaccharidosis type III A (Sanfilippo A syndrome)

Title	[Poster No.] Presented on
Non-clinical evaluation of a blood-brain barrier-penetrable N-sulfoglucosamine sulfohydrolase in a mouse model of Mucopolysaccharidosis IIIA	[252] February 11th 14:30-15:30 EST

Development code: JR-446, BBB-penetrating α-N-acetylglucosaminidase (rDNA origin)

Indication: Mucopolysaccharidosis type III B (Sanfilippo B syndrome)

Title	[Poster No.] Presented on
Non-clinical evaluation of a blood-brain barrier-penetrable α-N-acetylglucosaminidase in a mouse model of mucopolysaccharidosis IIIB	[103] February 8th 14:30-15:30 EST

WORLDSymposium™ attendees who would like more information about JCR Pharmaceuticals can visit https://congress.jcrpharm.co.jp/.

About the Annual WORLDSymposium™



The WORLDSymposium™ is designed for basic, translational and clinical researchers, patient advocacy groups, clinicians, and all others who are interested in learning more about the latest discoveries related to lysosomal diseases and the clinical investigation of these advances. For additional information on the 17th Annual WORLDSymposium2021™, please visit https://worldsymposia.org/.

[About JCR Pharmaceuticals]

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 leveraging our expertise in manufacturing and R&D to advance first-in-class medicine. 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 syndrome), MPS II (Hunter syndrome), Pompe disease, and more. 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.

Contact:

Investors & Media:

JCR Pharmaceuticals Co., Ltd.

Corporate Communications

ir-info@jcrpharm.co.jp

###