NEWS RELEASE

JCR Pharmaceuticals Announces 52-Week Interim Data from its Global Phase I/II Study of JR-171 in Individuals with Mucopolysaccharidosis Type I (MPS I)

9/29/2023

- JCR to Host Webinar on October 4, 2023, to Present Further Details -

HYOGO, Japan--(BUSINESS WIRE)-- **JCR Pharmaceuticals Co., Ltd.** (TSE 4552; Chairman and President: Shin Ashida; "JCR") announced today key results from the 52-week interim data of its global phase I/II study with JR-171 (INN: lepunafusp alfa) in individuals with mucopolysaccharidosis type I (MPS I, also known as Hurler, Hurler-Scheie and Scheie syndrome). JR-171 is a blood brain-barrier ("BBB")-penetrating form of recombinant α-L-iduronidase that was developed using JCR's proprietary J-Brain Cargo® technology. There are no approved therapies that cross the BBB and address the central nervous system (CNS) symptoms for individuals with MPS I.

The overall safety data concluded that JR-171 is suitable for the long-term treatment of individuals with MPS I. Although treatment duration and the number of individuals enrolled were not intended to demonstrate an effect on CNS symptoms, preliminary data suggest that JR-171 may provide a beneficial effect on neurological disease burden in symptomatic individuals. Somatic biomarkers remained stable in individuals previously exposed to α-L-iduronidase and were significantly reduced in the one individual who was treatment-naïve. This indicates that JR-171 provides somatic disease control comparable to somatic enzyme replacement therapy (ERT). Concentrations of substrate in the cerebrospinal fluid, which may indicate an effect of substrate reduction in the CNS, were significantly reduced in all patients.

"It is encouraging to observe additional changes in communication, executive functioning, and other aspects important in daily life for those individuals treated with JR-171, in addition to achieving the primary and secondary endpoints," said Paul Harmatz, MD, investigator at the UCSF Benioff Children's Hospital Oakland. "Based on these

data, JR-171 has the potential to treat both the somatic and neurological symptoms of individuals with MPS I, which is ultimately what is needed."

JCR Pharmaceuticals plans to host a webinar on October 4, 2023, to present further details of the 52-week results of the clinical study. To register for the webinar, please use the following link **https://us06web.zoom.us/webinar/register/WN_vLm-1crCQRqIA5gvk0ArkQ**.

There is no impact on the consolidated business results for this fiscal year ending on March 31, 2024, related to the matter.

About JR-171

JR-171 is a recombinant fusion protein of an antibody against the human transferrin receptor and α-L-iduronidase, the enzyme that is missing or malfunctioning in subjects with MPS I. By crossing the blood brain-barrier ("BBB") through transferrin receptor mediated transcytosis it is expected to be effective against central nervous system ("CNS") signs and symptoms of the disease thereby addressing a significant unmet need for the treatment of MPS I. JR-171 previously was granted Fast Track designation by the US Food and Drug Administration (FDA).

The phase I/II clinical study is being conducted at several centers in Brazil, Japan and the United States and is composed of two parts: Part one of the open-label study is a 4-week dose-escalation study in adult individuals with MPS I, while part 2 compares two doses of JR-171 in pediatric and adult individuals with MPS I. The main objectives of the phase I/II clinical study are to establish the safety of chronic dosing of JR-171 in individuals with MPS I, the pharmacokinetics of JR-171, to investigate somatic disease control based on biomarkers and somatic endpoints, and to explore early signs of efficacy on central nervous signs and symptoms of MPS I.

At WORLDSympoisumTM 2023 in February 2023, JCR already presented that the reduction of cerebrospinal fluid biomarker was observed in all patients, and biomarkers in serum and urine decreased (in treatment-naïve subjects) or were stable (in enzyme replacement therapy ("ERT")-experienced subjects) upon treatment with JR-171 for 12 weeks.

There were no drug-related serious adverse events (SAEs) reported during the study. Drug-related adverse events (AEs) and formation of anti-drug antibodies were comparable to profiles typically observed with somatic ERT. There were no gross differences in the safety profile of JR-171 at either dose level tested.

About the J-Brain Cargo® Platform Technology

JCR Pharmaceuticals has developed a proprietary BBB-penetrating technology J-Brain Cargo®, to bring

biotherapeutics into the CNS. The first drug developed based on this technology and approved in Japan for the treatment of MPS II (mucopolysaccharidosis type II) is IZGARGO® (INN: pabinafusp alfa). Based on the same platform technology, JR-171 is the second program advancing into global clinical stage. JCR intends to start clinical trials on five additional programs from its LSD pipeline by 2028.

About MPS I (Hurler, Hurler-Scheie, Scheie syndrome)

MPS I is an autosomal recessive lysosomal storage disorders ("LSD") caused by a deficiency of α-L-iduronidase, an enzyme that breaks down glycosaminoglycans (mucopolysaccharides) in the body. The current worldwide prevalence of MPS I is estimated to be approximately 3,000 (according to JCR internal research). MPS I gives rise to a wide range of somatic and neurological symptoms. A major limitation of current ERT is that it does not address CNS symptoms because of the enzyme's inability cross the BBB. MPS I is the only LSD in which hematopoietic stem cell transplantation ("HSCT") is part of the standard of case in the severe form of the disease. Significant unmet medical need persists in all forms of MPS I.

About JCR Pharmaceuticals Co., Ltd.

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 48-year legacy in Japan while expanding our global footprint into the US, Europe, and Latin America. We improve patients' lives by applying our scientific expertise and unique technologies to research, develop, and deliver next-generation therapies. 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, Hurler-Scheie and Scheie syndrome), MPS II (Hunter syndrome), MPS III A and B (Sanfilippo syndrome type A and B), and more. JCR strives to expand the possibilities for patients while accelerating medical advancement at a global level. 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.

Investors & Media:
JCR Pharmaceuticals Co., Ltd.
Corporate Communications
ir-info@jp.jcrpharm.com

Source: JCR Pharmaceuticals Co., Ltd.