



June 5, 2023
JCR Pharmaceuticals Co., Ltd.

Translation

JCR Receives the 48th Inoue Harushige Prize for Therapeutics for Lysosomal Storage Disorders with an Original BBB Penetrating Technology

June 5, 2023 -- JCR Pharmaceuticals Co., Ltd. (TSE 4552; Chairman, President and CED: Shin Ashida; "JCR") announced that it has received the 48th Inoue Harushige Prize for the development of biopharmaceuticals through the world's first blood-brain barrier (BBB) technology together with William M. Pardridge, M.D., Distinguished Professor Emeritus at UCLA.

The Inoue Harushige Prize is an award which was created by the Research Development Corporation of Japan, one of the predecessors of the Japan Science and Technology Agency (JST), and is given to researchers and corporations for outstanding technology in Japan that a company has developed and commercialized using original research from a university, research institute, or similar entity. The award ceremony will be held on July 20th.

J-Brain Cargo[®] is an innovative technology that enables the delivery of drugs to the brain across existing barriers in the brain. The brain is protected from many toxic, noxious or infectious agents by a barrier called the BBB, which also prevents biopharmaceutical products from actively entering the brain. Professor Pardridge, M.D. and his associates proposed a method called "Trojan Horse Technology*" that utilizes a system which is innate to humans. Subsequently, JCR utilized this method as a drug delivery technology to achieve effective BBB penetration and succeeded at developing J-Brain Cargo[®] which is based on anti-human transferrin receptor antibodies.

*This means that it deceives the natural defense functions of the BBB, similar to the story of the Trojan horse in Greek mythology.

Lysosomal storage disorders ("LSDs") often present central nervous system manifestations that do not improve through conventional enzyme replacement therapy. IZCARGO[®] I.V. infusion 10mg (JAN: Pabinafusp Alfa (Genetical Recombination)) was developed for the treatment of mucopolysaccharidosis II (MPS II, or Hunter syndrome), using J-Brain Cargo[®], and was approved for manufacturing and marketing in March 2021, and launched in Japan in May of the same year, making it the world's first biopharmaceutical product to actively cross the BBB. A Global phase III clinical trial is currently being conducted to bring pabinafusp alfa to global approval.

JCR believes that receiving this award is a recognition of J-Brain Cargo[®] as a core technology that can be applied to a variety of central nervous system diseases, and that there are high expectations for the expansion of its application to a wide range of diseases in the future.

Shin Ashida, Chairman, President and CEO of JCR Pharma, commented "I feel honored that JCR receives such a distinguished award. JCR's J-Brain Cargo[®] is a revolutionary technology which

offers new potential for various diseases that were previously untreatable. Having built foundational “R&D” and “Manufacturing” capabilities, we aim to deliver value only JCR can provide for as many patients as possible as quickly as we can.”

William M. Partridge, M.D., Distinguished Professor Emeritus at UCLA, issued the following statement. “The leadership of JCR Pharma in the development of novel biologic drugs for the brain is now recognized by their selection as the 2023 recipient of the Inoue Harushige Prize. JCR is the first worldwide pharmaceutical company to receive market approval of a biologic drug for a brain disorder, wherein the biologic is re-engineered to enable drug transport across the blood-brain barrier (BBB). The biologic drug is the lysosomal enzyme that is mutated in an inborn error of metabolism called Hunter syndrome. Similar to other biologic drugs, this enzyme does not cross the BBB and enzyme replacement therapy of Hunter syndrome does not treat the brain. In the re-engineering process, the enzyme is genetically fused to a BBB Trojan horse. The JCR BBB Trojan horse is a monoclonal antibody that binds a receptor on the BBB, which triggers receptor-mediated transport (RMT) of the antibody across the BBB. The fusion protein of the Trojan horse antibody and the enzyme also traverses the BBB via this RMT pathway to enable treatment of the brain of patients born with Hunter syndrome. The JCR technology for solving the BBB drug delivery problem can be expanded to virtually any biologic drug, which will enable future treatment of common brain disorders, including Alzheimer’s disease, Parkinson’s disease, brain cancer, stroke, and other serious conditions of the brain.”

JCR will continue to deliver medicine that only we can provide for as many patients as possible and their families through R&D and manufacturing.

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), Pompe disease, 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.

Contact:

Investors & Media:

JCR Pharmaceuticals Co., Ltd.

Corporate Communications

ir-info@jp.jcrpharm.com

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