News Release

October 23, 2024

JCR Pharmaceuticals Co., Ltd.

JCR Pharmaceuticals Presents Preclinical Gene Therapy Research at the European Society of Gene and Cell Therapy 31st Annual Congress

- Preclinical Data Demonstrate Potential to Deliver an AAV Gene Therapy Using JCR's Proprietary J-Brain Cargo® Technology to Treat Central Nervous System Diseases -

Hyogo, Japan -- October 23, 2024 -- JCR Pharmaceuticals Co., Ltd. (TSE 4552; Chairman and President: Shin Ashida; "JCR") announced today that the Company presented preclinical data from its novel adeno-associated virus (AAV) gene therapy research programs in a poster session at the European Society of Gene and Cell Therapy (ESGCT) 31st Annual Congress, being held October 22-25, 2024, in Rome, Italy. JCR leverages its proprietary J-Brain Cargo® (JBC) technology for the delivery of adeno-associated virus (AAV) gene therapy to the body and the brain to potentially address the central nervous system (CNS) symptoms by crossing the blood-brain barrier (BBB).

"These findings illustrate the potential for applying our proprietary JBC technology as a vehicle to deliver gene therapy across the blood-brain barrier to potentially treat diseases in the central nervous system," said Hiroyuki Sonoda, Ph.D., Director, Senior Managing Executive Officer, and Executive Director, Research Division at JCR Pharmaceuticals. "This approach has the potential to unlock new therapeutic options for conditions that have historically been challenging to treat. The results from these preclinical studies indicate that the uptake of the gene therapy using our proprietary JBC technology was substantially higher than with AAV9 and a reduction in tropism to the liver, thereby enhancing the safety of AAVs for human-use. We are committed to advancing innovative solutions that address unmet medical needs, and this research marks a crucial step forward."

JCR showcased the following presentation:

Incorporation of transferrin receptor binder into AAV enables efficient brain delivery for treatment of genetic CNS diseases (P0133)
Presenter: Yuhei Ashida (JCR Pharmaceuticals)
The AAV vector leveraging the JBC technology transduced cells in the CNS more efficiently than a conventional AAV9 vector in mice and monkeys. When the modified vector was applied to mouse models of genetic CNS diseases, efficient reduction of toxic substances, histopathological improvements of the brain, improvements of behavioral tests, and greatly improved survival were observed. In addition, a JBC-AAV molecule with additional modifications showed dramatically reduced liver transduction. These results suggest that JCR's JBC-AAV has the potential to be a promising tool for gene therapy of CNS diseases.

Researchers noted the potential advantages of using JBC technology to deliver the AAV gene therapy to the CNS. The JBC-AAV capsid has versatility to fine-tune affinity and uptake. In addition, the capsid has a simple manufacturing scheme and stable production yields.

About the European Society of Gene and Cell Therapy (ESGCT)

Established in 1992, the European Society of Gene and Cell Therapy (ESGCT) seeks to support scientists and clinicians working in the fields of gene and cell therapy and to promote awareness

and understanding of gene and cell therapy and the vast amount of related research in Europe. For more information, please visit https://www.esgct.eu/.

About the J-Brain Cargo® Platform Technology

JCR Pharmaceuticals has developed a proprietary blood-brain barrier (BBB)-penetrating technology, J-Brain Cargo®, to bring biotherapeutics into the central nervous system (CNS). The first drug developed based on this technology is IZCARGO® (INN: pabinafusp alfa) and was approved in Japan for the treatment of a lysosomal storage disorder.

About JCR Pharmaceuticals Co., Ltd.

JCR Pharmaceuticals Co., Ltd. (TSE 4552) is a global specialty pharmaceuticals company that is expanding possibilities for people with rare and genetic diseases worldwide. We continue to build upon our 49-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, MPS II (Hunter syndrome), 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, MPS IIIA 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. 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.

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