News Release

May 15, 2025

JCR Pharmaceuticals Co., Ltd.

JCR Pharmaceuticals Presents Preclinical Gene Therapy Data that Demonstrates Promising CNS Uptake at American Society of Gene and Cell Therapy 28th Annual Meeting

- Research Highlights the Potential of JCR's Proprietary J-Brain Cargo® Technology to Facilitate Efficient Delivery of an AAV Gene Therapy to the Central Nervous System –

Hyogo, Japan – **May 15, 2025 –** JCR Pharmaceuticals Co., Ltd. (TSE 4552; "JCR") announced today that the Company presented preclinical data about its novel adeno-associated virus (AAV) gene therapy research programs at the American Society of Gene and Cell Therapy (ASGCT) 28th Annual Meeting, being held May 13-17, 2025, in New Orleans, LA. In an oral presentation, the JCR researcher reported that the Company's proprietary J-Brain Cargo® (JBC) technology enables the efficient delivery of an adeno-associated virus (AAV) gene therapy across the blood-brain barrier (BBB) and into the central nervous system (CNS) in mice, monkeys and several animal models of CNS diseases.

We have successfully developed JUST-AAV, a novel adeno-associated virus (AAV) vector platform technology designed to enhance targeted delivery and reduce liver tropism, thereby improving safety and efficacy of AAV-based gene delivery technologies to the CNS. JUST-AAV encompasses a range of vector types optimized for various target tissues, including liver-sparing, muscle-targeting, and brain-targeting variants. This proprietary technology holds significant promise for advancing the field of AAV-based gene therapy.

"These findings represent a significant advancement toward new treatments for previously challenging CNS diseases," said Hiroyuki Sonoda, Ph.D., Director, Senior Managing Executive Officer, and Executive Director, Research Division at JCR Pharmaceuticals. "Our preclinical data demonstrate that our brain-targeting JUST-AAV technology delivers therapeutic agents to the central nervous system far more efficiently than conventional AAV9, while significantly reducing liver accumulation. This enhanced safety profile greatly increases the potential for clinical translation. This research is a crucial step in our ongoing commitment to developing innovative solutions for unmet medical needs."

JCR showcased the following presentation:

Incorporation of transferrin receptor binder and surface mutations into AAV enables efficient brain delivery and reduced liver tropism
Presenter: Yuhei Ashida, (JCR Pharmaceuticals)

Researchers successfully created a brain-targeting AAV vector (brain-targeting JUST-AAV) by incorporating a miniaturized antibody that binds to the transferrin receptor, into the AAV capsid. Furthermore, unique modifications to the AAV capsid sequence significantly reduced AAV vector accumulation in the liver, a known source of adverse effects. In mouse studies, the JUST-AAV vector achieved 77-fold higher expression of green fluorescent protein (GFP) in the brain

compared to AAV9, while reducing the tropism to the liver by 99%. To further enhance brain targeting and blood-brain barrier permeability, the researchers incorporated additional molecules that bind to receptors other than the transferrin receptor into the AAV capsid. In monkey studies, this bispecific vector demonstrated an improved gene delivery efficiency to the brain by several orders of magnitude compared with AAV9, while reducing infection in potentially problematic tissues such as the liver and dorsal root ganglia by more than 90%.

Application of JUST-AAV to a mouse model of neuronal ceroid lipofuscinosis resulted in the disappearance of symptoms such as seizures and prolonged lifespan to an extent of functional cure. These results suggest that the newly developed JUST-AAV technology offers the potential for safer and more efficient gene therapy than conventional gene delivery vectors.

About the American Society of Gene and Cell Therapy (ASGCT)

The American Society of Gene and Cell Therapy (ASGCT) is the primary professional membership organization for gene and cell therapy. The Society's members are scientists, physicians, patient advocates, and other professionals. The mission of the ASGCT is to advance knowledge, awareness, and education, leading to the discovery and clinical application of genetic and cellular therapies to alleviate human disease. For more information, please visit www.asgct.org.

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.

JUST-AAV

JUST-AAV is a proprietary platform technology that utilizes modified adeno-associated virus (AAV) vectors. The technology entails insertion of miniaturized antibodies against receptors on selected tissues, organs or the blood-brain barrier onto the capsid surface, enhancing targeted delivery to those tissues and organs. Further capsid modifications minimize off-target effects and improve safety. The name is derived from "**J**CR" "**U**ltimate destination of organ" "**S**afeguarding against off-target delivery" and "**T**ransformative technology" reflecting its potential for broad application across various diseases.

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 50-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. For more information, please visit https://www.jcrpharm.co.jp/en/site/en/index.html

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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