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JCR Pharmaceuticals Co., Ltd.

JCR Pharmaceuticals Enhances Delivery of AAV Gene Therapy to Central Nervous System and Muscle with Novel Capsid Engineering Platform Technology

- Oral Presentation at ESGCT Highlights Potential of JCR's Proprietary JUST-AAV Platform to Facilitate More Efficient Delivery While Reducing Liver Tropism -

Hyogo, Japan – October 7, 2025 – [JCR Pharmaceuticals Co., Ltd.](#) (TSE 4552; “JCR”), a global specialty biopharmaceutical company dedicated to developing therapies for rare and genetic diseases, today presented non-clinical data demonstrating the ability of its proprietary JUST-AAV capsid engineering platform to achieve efficient delivery of adeno-associated virus (AAV) gene therapy to the central nervous system (CNS) and muscle while reducing liver exposure. The data, presented at the European Society of Gene and Cell Therapy (ESGCT) 32nd Annual Congress, in Seville, Spain, showcase the platform's potential to overcome longstanding challenges in treating CNS and muscle disorders.

In an oral presentation, JCR researchers reported that the JUST-AAV platform enables the efficient delivery of JUST-AAV into the muscle in mice and monkeys, when compared to the conventional AAV. The JUST-AAV was modified to improve the distribution into the muscle. It achieved higher transduction in muscle, while it showed reduced tropism drastically in the liver. It indicates the platform's potential to improve the safety and efficacy of AAV-based gene delivery technologies to target tissues.

“Our JUST-AAV capsid engineering platform points the way toward development of new, more precisely targeted gene therapies for central nervous system diseases that have long been considered out of reach,” said Hiroyuki Sonoda, Ph.D., Director, Senior Managing Executive Officer, and Executive Director of the Research Division at JCR Pharmaceuticals. “The data presented at ESGCT demonstrate that the platform delivers therapeutic agents to the brain and muscle far more efficiently than conventional AAV9 capsids, while limiting accumulation in the liver. This research is a crucial step in our ongoing commitment to developing innovative solutions for complex healthcare challenges.”

JUST-AAV encompasses a range of vector types optimized for various target tissues, including liver-sparing, muscle-targeting, and brain-targeting variants. Its target specificity is enhanced via J-Brain Cargo® that shows improved CNS transduction when incorporated into an AAV capsid, compared to AAV9, based on results from non-clinical studies in mice and monkeys. JUST-AAV holds significant promise for advancing the field of AAV-based gene therapy.

JCR showcased the following presentation at the ESGCT Congress:

Generation of a novel capsid engineering platform enhances CNS and muscle tropism of AAV vectors while reducing tropism to the liver

Presenter: Shunsuke Iizuka, Ph.D., Associate Senior Scientist, JCR Pharmaceuticals

Dr. Iizuka reported on the generation of a novel series of AAV capsids allowing enhanced delivery to the CNS and/or skeletal muscle, heart, and diaphragm, while decreasing liver tropism via separate capsid mutations using the proprietary JUST-AAV platform technology. He and his

colleagues identified a peptide that, when incorporated onto the surface of the AAV capsid, enhanced muscular uptake. Validation of transgene expression using Green Fluorescent Protein (GFP) yielded a 15-fold increase in GFP messenger RNA (mRNA) levels in the quadriceps and heart in mice. Further screening efforts led to the identification of new muscle tropic binders to further improve transduction efficiency. Through screening a library of antibody fragments, they identified the binder that, when incorporated into the AAV capsid, improved muscle transduction by a factor of 2- to 3-fold compared to previous muscle-targeted AAV capsids. Characterization of the new binder revealed that it has equal affinity for human, monkey, and mouse receptors.

They incorporated this novel muscle-targeting binder along with a liver de-targeting mutation on the AAV capsid, which was then evaluated in mice. This muscle-targeting JUST-AAV demonstrated 28- and 52-times higher transduction efficiencies in quadriceps and heart compared to AAV9. Additionally, this AAV showed dramatically reduced liver transduction by 160-fold compared to AAV9. Furthermore, they successfully developed dual-targeting JUST-AAV by incorporating both muscle- and brain-targeting binder, J-Brain Cargo®, on the single capsid. This vector represented improved transduction in both brain and muscle tissues, achieving levels comparable to AAVs with single binders.

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. The 32nd Annual Congress of the ESGCT is being held in Seville, Spain from October 7-10, 2025. For more information, please visit www.esgct.eu.

About the JUST-AAV Platform Technology

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 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), which is approved in Japan for the treatment of a lysosomal storage disorder (LSD). With J-Brain Cargo®, JCR seeks to address the unresolved clinical challenges of LSDs by delivering the enzyme to both the body and the brain.

About JCR Pharmaceuticals Co., Ltd.

JCR Pharmaceuticals Co., Ltd. (TSE 4552) is a global specialty pharmaceutical company that develops treatments that go beyond rare diseases to solve the world's most complex healthcare challenges. We continue to build upon our 50-year legacy in Japan while expanding our global footprint into the U.S., 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. Our core values – Putting people first, Forging our own path, Always advancing, and Committed to excellence – mean that the work we do benefits all our stakeholders, including partners, patients and employees. We strive to expand the possibilities

for patients while accelerating medical advancement at a global level. For more information, please visit JCR's global website: <https://jcrpharm.com/>.

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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