Life is Rare

May 18, 2026

JCR Pharmaceuticals Co., Ltd.

JCR Pharmaceuticals Presents Preclinical Gene Therapy Data that Demonstrate Promising Central Nervous System Uptake at the American Society of Gene and Cell Therapy (ASGCT) 29th Annual Meeting

- Research Describes the Potential of JCR's Platform Technologies, Including JUST-AAV Gene Therapy, to Facilitate Delivery of Therapies to the Central Nervous System -

Hyogo, Japan – May 18, 2026 – JCR Pharmaceuticals Co., Ltd. (TSE 4552; "JCR"), a global specialty biopharmaceutical company dedicated to developing therapies for rare and genetic diseases, announced today that it presented preclinical data from its novel platform technologies, including JUST-AAV gene therapy, in oral and poster sessions at the American Society of Gene and Cell Therapy (ASGCT) 29th Annual Meeting, being held May 11-15, 2026, in Boston, Massachusetts. Additionally, Alexion, AstraZeneca Rare Disease, presented preclinical data from a research collaboration that applies JCR's JUST-AAV technology in an oral session.

JUST-AAV is JCR's novel adeno-associated vector (AAV) vector platform technology, which is under preclinical investigation, and is designed to enhance targeted delivery to the central nervous system (CNS) and/or muscle and reduce liver tropism, aiming to improve safety and efficacy of AAV-based gene delivery technologies. 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 promise for advancing the field of AAV-based gene therapy.

"The presented preclinical results demonstrate that JCR's novel capsid platform is able to deliver therapeutic agents to the central nervous system more efficiently than conventional AAV9, while reducing liver accumulation," said Hiroyuki Sonoda, Ph.D., President and Chief Scientific Officer at JCR Pharmaceuticals. "These data represent an advancement toward potential new treatment options for previously challenging CNS diseases. This research is an important step forward in our ongoing commitment to developing innovative solutions for complex healthcare challenges, including neurodegenerative disorders."

Development of an AAV-Based Gene Therapy for GM1 Gangliosidosis Using a Transferrin Receptor Antibody–Fused Enzyme with Markedly Enhanced Therapeutic Efficacy (Presentation Number: 391)

Presenter: Saki Matsushima, Ph.D. (The Jikei University School of Medicine, Division of Gene Therapy, Research Center for Medical Sciences, Tokyo, Japan)

Researchers reported on preclinical data from an AAV9 gene therapy encoding a transferrin receptor (TfR)-targeted, blood-brain barrier (BBB)-penetrable lysosomal enzyme, which is being investigated for GM1 gangliosidosis. Researchers used a plasmid encoding β-galactosidase fused to an antibody-derived TfR-binding domain to enable receptor-mediated BBB transport once the transgene is expressed in the treated animals. They demonstrated proof-of-concept in a mouse model of GM1 gangliosidosis using a mouse TfR-targeting construct.

Intravenous administration of the liver-specific AAV vector resulted in robust hepatic expression and high circulating levels of the TfR-targeted β-galactosidase, enabling efficient distribution to peripheral tissues and the CNS. In the animals, the mouse TfR-targeting construct markedly reduced GM1 ganglioside accumulation in the brain, improved neurological function, and significantly extended survival. Additionally, in GM1 mice expressing human TfR, the human TfR-targeted enzyme demonstrated comparable biodistribution, CNS penetration, and therapeutic efficacy. Researchers did not observe any apparent toxicity associated with hepatic expression or systemic enzyme exposure.

These results demonstrate that AAV gene therapy expressing a TfR-targeted, BBB-penetrable

lysosomal enzyme enables robust CNS correction in GM1 gangliosidosis. The successful evaluation of a human TfR-targeted construct in a humanized disease model, leveraging a clinically validated BBB transport strategy, provides strong translational support for first-in-human clinical trials.

Long-Term Efficacy and Neuroprotection by Systemically Administered, CNS-Targeting AAV Capsids in Mouse Models of Neuronal Ceroid Lipofuscinosis (CLN1 and CLN2) (Poster Number: 3460)
Lead Author: Tomoki Hirashima (JCR Pharmaceuticals)

JCR researchers reported on the long-term efficacy of JUST-AAV-mediated gene therapy based on preclinical data from mouse models of neuronal ceroid lipofuscinosis (CLN1 and CLN2) compared with recombinant enzyme replacement therapy and conventional AAV approaches. In the CLN1 (PPT1 deficiency) study, researchers assessed five groups across survival, motor function, cognition, neuropathology, and retinal integrity. Mice in the disease control and recombinant PPT1 group showed no survival benefit, with death occurring between weeks 31–41. Mice treated with conventional AAV9-PPT1 extended survival to weeks 53–65. In contrast, mice treated with JCR's investigational therapy, JUST-AAV-PPT1, had an almost comparable life span to healthy control animals. The proportion of SCMAS-, CD68-, and GFAP-positive areas—markers of lysosomal storage and inflammation—were markedly reduced in the JUST-AAV-PPT1 group, indicating suppressed neuroinflammation and microglial activation. Brain extracts confirmed elevated PPT1 enzymatic activity in JUST-AAV–treated mice. Researchers noted that preservation of locomotor function at 54 weeks of age was indistinguishable from healthy wildtype animals. Measurement of the retinal ganglion cell layer thickness revealed a significant loss in thickness in diseased animals, while retinal thickness was preserved in the JUST-AAV-PPT1–treated mice.

In the CLN2 (TPP1 deficiency) study, systemic administration of JCR's investigational therapy, JUST-AAV-TPP1, led to a comparably pronounced therapeutic effect regarding survival compared with healthy control mice. Post-mortem analyses confirmed elevated TPP1 activity in brain tissue, supporting robust CNS delivery and sustained therapeutic benefit. Researchers concluded that JUST-AAV–mediated gene therapy significantly extended lifespan and improved pathogenic phenotypic changes in CLN1 and CLN2 mouse models, indicating the ability to efficiently deliver the transgene to the CNS upon systemic administration. Based on these preclinical results, the JUST-AAV technology holds strong potential as a next-generation gene therapy platform for lysosomal storage disorders and other neurodegenerative diseases.

Alexion, AstraZeneca Rare Disease presented the following:

AAV Capsid Presenting a Miniaturized Anti–Transferrin Receptor Antibody Enables Broad CNS, Liver-detargeted Biodistribution (Presentation Number: 427)

Researchers reported on preclinical data that evaluated a TfR-targeted JUST-AAV capsid, based on the JUST-AAV technology, that expresses a miniaturized anti–TfR receptor binder on the surface of the capsid and incorporates sequence modifications to minimize liver exposure. Following a single intravenous dose in mouse models and non-human primates, the capsid enabled broad CNS biodistribution, while increasing brain-to-liver exposure ratios relative to AAV9. Analyses demonstrated dose-dependent transgene expression with favorable tolerability. Researchers concluded that these data support a translational platform for broad brain distribution with reduced liver exposure.

The data referenced are from preclinical studies. Study limitations were not discussed.

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 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 liver tropism and potentially mitigate hepatoxicity, which is a commonly observed safety concern. 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 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/.

Contact:

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

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