



Life is Rare

May 11, 2026

JCR Pharmaceuticals Co., Ltd.

## **JCR Pharmaceuticals to Present at the American Society of Gene and Cell Therapy (ASGCT) 29<sup>th</sup> Annual Meeting**

**Hyogo, Japan – May 11, 2026 – [JCR Pharmaceuticals Co., Ltd.](https://www.jcr-pharm.com)** (TSE 4552; “JCR”), a global specialty biopharmaceutical company dedicated to developing therapies for rare and genetic diseases, announced today that it will present 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) 29<sup>th</sup> Annual Meeting, being held May 11-15, 2026, in Boston, Massachusetts. Additionally, Alexion, AstraZeneca Rare Disease, will present preclinical data from a research collaboration that applies the JUST-AAV technology in an oral session.

The details of the presentations are listed below, and the full program can be found on the ASGCT congress website at <https://annualmeeting.asgct.org/>.

**Title:** Development of an AAV-Based Gene Therapy for GM1 Gangliosidosis Using a Transferrin Receptor Antibody–Fused Enzyme with Markedly Enhanced Therapeutic Efficacy

**Session:** Neurologic Diseases VI

**Presentation Number:** 391

**Date/Time:** Thursday, May 14, 4:45 PM - 5:00 PM ET

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

**Title:** Long-Term Efficacy and Neuroprotection by Systemically Administered, CNS-Targeting AAV Capsids in Mouse Models of Neuronal Ceroid Lipofuscinosis (CLN1 and CLN2)

**Session:** Poster Reception

**Poster Number:** 3460

**Date/Time:** Thursday, May 14, 5:00 PM – 6:30 PM ET

**Lead Author:** Tomoki Hirashima (JCR Pharmaceuticals)

**Title:** AAV Capsid Presenting a Miniaturized Anti–Transferrin Receptor Antibody Enables Broad CNS, Liver-detargeted Biodistribution

**Session:** AAV Capsid Engineering IV

**Presentation Number:** 427

**Date/Time:** Friday, May 15, 9:15 AM - 9:30 AM ET

### **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](http://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 hepatotoxicity, 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](mailto:ir-info@jp.jcrpharm.com)

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