



Life is Rare

September 30, 2025

JCR Pharmaceuticals Co., Ltd.

JCR Pharmaceuticals to Present at the European Society of Gene and Cell Therapy (ESGCT) 32nd Annual Congress

Hyogo, Japan – September 30, 2025 -- [JCR Pharmaceuticals Co., Ltd.](https://www.jcr-pharm.co.jp/en) (TSE 4552; “JCR”), a global specialty biopharmaceutical company dedicated to developing therapies for rare and genetic diseases, announced today that it will present non-clinical data from its novel JUST-AAV gene therapy platform technology in an oral session at the European Society of Gene and Cell Therapy (ESGCT) 32nd Annual Congress, being held October 7-10, 2025, in Seville, Spain.

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 a new muscle-targeting binder that shows cross-species affinity and improved muscle transduction when incorporated into an AAV capsid, compared to AAV9. JUST-AAV thus holds significant promise for advancing the field of AAV-based gene therapy.

The oral presentation details are listed below, and the full program can be found on the ESGCT congress website at www.esgctcongress.com.

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

Oral Presentation Number: OR009

Date: October 7, 2025, 5:00-7:30pm CEST

Presenter: Shunsuke Iizuka, Ph.D., JCR Pharmaceuticals

About 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 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/>.

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