



May 8, 2025

JCR Pharmaceuticals Co., Ltd.

JCR Pharmaceuticals to Present at the American Society of Gene and Cell Therapy 28th Annual Meeting

Hyogo, Japan – May 8, 2025 – [JCR Pharmaceuticals Co., Ltd.](#) (TSE 4552; “JCR”) announced today that it will present updated preclinical data from its proprietary J-Brain Cargo[®]-applied adeno-associated virus (AAV) vector gene therapy non-clinical research programs in an oral abstract session at the American Society of Gene and Cell Therapy (ASGCT) 28th Annual Meeting, being held May 13-17, 2025, in New Orleans, LA.

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

Title: Incorporation of transferrin receptor binder and surface mutations into AAV enables efficient brain delivery and reduced liver tropism

Session: AAV Gene Transfer (A): Crossing the Blood-Brain Barrier

Abstract Number: 92

Date/Time: Wednesday, May 14, 2:30-2:45pm CT

Presenter: Yuhei Ashida, Associate Senior Scientist, at JCR Pharmaceuticals

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.

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. Our core values – reliability, confidence, and persistence – benefit all our

stakeholders, including employees, partners, and patients. For more information, please visit <https://www.jcrpharm.co.jp/en/site/en/>

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