



August 28, 2025

JCR Pharmaceuticals Co., Ltd.

JCR Pharmaceuticals to Present at the 15th International Congress of Inborn Errors of Metabolism (ICIEM) 2025

- Data to be showcased in one oral presentation and four poster presentations -

Hyogo, Japan – August 28, 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 data at the 15th International Congress of Inborn Errors of Metabolism (ICIEM) 2025, held September 2-6, 2025, in Kyoto, Japan. The presentations will demonstrate the potential benefits of the investigational therapies in JCR’s development pipeline and of J-Brain Cargo®, JCR’s proprietary technology that delivers medicine across the blood-brain barrier (BBB) for the treatment of lysosomal storage disorders and neurodegenerative disorders.

Details of the presentations are as follows:

Poster Presentation Title: *Therapeutic effect of JR-141 (pabinafusp alfa) on cardiovascular system in a mouse model of mucopolysaccharidosis type II*

Date/Time: Wednesday, September 3, 2025, 18:00-19:00 JST

Presenter: Kenta Arisumi (JCR Pharmaceuticals)

Poster Number: P-311

Abstract Number: 11189

Poster Presentation Title: *Nonclinical pharmacology of JR-446, a novel blood-brain barrier penetrant α -N-acetylglucosaminidase*

Date/Time: Wednesday, September 3, 2025, 18:00-19:00 JST

Presenter: Jun Ito (JCR Pharmaceuticals)

Poster Number: BP-15

Abstract Number: 11217

Poster Presentation Title: *Long-term safety of pabinafusp alfa in patients with mucopolysaccharidosis type II: interim 5-year data from a clinical trial in Japan*

Date/Time: Thursday, September 4, 2025, 17:30-18:30 JST

Presenter: Norio Sakai, M.D., Ph.D. (Center for Promoting Treatment of Intractable Diseases, ISEIKAI International General Hospital, Osaka, Japan)

Poster Number: P-306

Abstract Number: 11355

Oral Presentation Title: *Long-term (up to 5 years) efficacy of pabinafusp alfa on neurocognition in patients with mucopolysaccharidosis type II – a pooled, post hoc analysis of clinical trials*

Date/Time: Friday, September 5, 2025, 13:30-15:00 JST

Presenter: Roberto Giugliani, M.D., Ph.D. (Federal University of Rio Grande do Sul, Brazil)

Oral Presentation: 24

Abstract Number: 11339

e-Poster Title: *Post-marketing surveillance of pabinafusp alfa for the treatment of mucopolysaccharidosis type II: an interim report up to 4 years*

Lead Author: Shungo Okamoto, M.D. (Department of Pediatrics, Osaka Metropolitan University Graduate School of Medicine, Osaka, Japan)

Abstract Number: 11285

About the International Congress of Inborn Errors of Metabolism (ICIEM)

The International Congress of Inborn Errors of Metabolism (ICIEM) is a major international conference held every four years, focusing on the study and treatment of inherited metabolic disorders. ICIEM conferences bring together researchers, clinicians, and other professionals to discuss the latest advancements in the field of inborn errors of metabolism.

About the J-Brain Cargo® Platform Technology

JCR Pharmaceuticals has developed a proprietary BBB-penetrating technology J-Brain Cargo® to bring biotherapeutics into the central nervous system. The first drug developed based on this technology was approved in Japan for a rare lysosomal storage disorder.

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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