Life is Rare

January 27, 2026

JCR Pharmaceuticals Co., Ltd.

JCR Pharmaceuticals to Present at the 22nd Annual WORLD*Symposium*™ 2026

- Data to be presented in four poster presentations -

Hyogo, Japan – **January 27, 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 will present data at the 22nd Annual WORLD*Symposium*™ 2026, held February 2-6, 2026, in San Diego, Calif. The poster 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 other neurodegenerative diseases.

Details of the presentations are as follows:

Presentation Title: *A transferrin receptor-targeted α-L-fucosidase, JR-471, reduced core-fucosylated glycoasparagine in the brain and preserved motor function in a murine model of Fucosidosis*
Date/Time: *February 3, 2026, 15:30-17:30 PST*
Presenter: *Tomomi Masuda, Ph.D. (JCR Pharmaceuticals)*
Poster Number: *246*

Presentation Title: *Sustained cognitive and adaptive behavior outcomes of long-term treatment with pabinafusp alfa in patients with severe or attenuated mucopolysaccharidosis type II*
Date/Time: *February 5, 2026, 15:30-17:30 PST*
Presenter: *Roberto Giugliani, M.D., Ph.D. (Federal University of Rio Grande do Sul, Brazil)*
Poster Number: *133*

Presentation Title: *Long-term somatic efficacy of pabinafusp alfa across a broad spectrum of age groups and phenotypes in patients with mucopolysaccharidosis type II*
Date/Time: *February 5, 2026, 15:30-17:30 PST*
Presenter: *Ana Maria Martins, M.D., Ph.D. (Federal University of São Paulo)*
Poster Number: *245*

Presentation Title: *Infusion rate adjustment in enzyme replacement therapy with pabinafusp alfa for mucopolysaccharidosis II*
Date/Time: *February 5, 2026, 15:30-17:30 PST*
Presenter: *Norio Sakai, M.D., Ph.D. (ISEIKAI International General Hospital, Osaka, Japan)*
Poster Number: *310*

The first presentation will spotlight JR-471, an investigational BBB-penetrating α-L-fucosidase (rDNA origin) enzyme replacement therapy (ERT) that JCR is developing for the treatment of people with fucosidosis.

The other three presentations will highlight JCR's lead product candidate, JR-141 (pabinafusp alfa, intravenous infusion), a recombinant fusion protein consisting of a humanized anti-transferrin receptor antibody and iduronate-2-sulfatase for the treatment of people with mucopolysaccharidosis type II (MPS II, or Hunter syndrome). The Ministry of Health, Labour and Welfare (MHLW) in Japan approved JR-141 under the brand name IZCARGO™ in March 2021 for a rare lysosomal storage disorder.

WORLD*Symposi*um™ attendees who would like to receive more information about JCR Pharmaceuticals can visit JCR's on-site conference booth (#407).

About the Annual WORLD*Symposium*™

The WORLD*Symposium*™ is designed for basic, translational and clinical researchers, patient advocacy groups, clinicians, and all others who are interested in learning more about the latest discoveries related to lysosomal diseases and the clinical investigation of these advances. For additional information on the 22nd Annual WORLD*Symposium*™, please visit https://worldsymposia.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), which is approved in Japan for the treatment of a lysosomal storage disorder (LSD). With J-Brain Cargo®, JCR seeks to address the unresolved clinical challenges of LSDs by delivering the enzyme to both the body and the brain.

About JR-471

JR-471 is a recombinant fusion protein of α-L-fucosidase and J-Brain Cargo®, JCR's proprietary blood-brain barrier (BBB)-penetrating technology. JCR and MEDIPAL HOLDINGS CORPORATION are developing JR-471 for the treatment of fucosidosis, which is currently in the pre-clinical stage.

About Pabinafusp Alfa

Pabinafusp alfa is a recombinant fusion protein of an antibody against the human transferrin receptor and iduronate-2-sulfatase, the enzyme that is missing or malfunctioning in subjects with Hunter syndrome. It incorporates J-Brain Cargo®, JCR's proprietary blood-brain barrier (BBB)-penetrating technology, to cross the BBB through transferrin receptor-mediated transcytosis, and its uptake into cells is mediated through the mannose-6-phosphate receptor. This novel mechanism of action is expected to make IZCARGO™ effective against the central nervous system (CNS) symptoms of Hunter syndrome.

In pre-clinical trials, JCR has confirmed both high-affinity binding of pabinafusp alfa to transferrin receptors and passage across the BBB into neuronal cells. In addition, JCR has confirmed enzyme uptake in various brain tissues. The company has also confirmed a reduction of substrate accumulation in the CNS and peripheral organs in an animal model of Hunter syndrome.[1,2]

In several clinical trials of pabinafusp alfa, JCR obtained evidence of reducing heparan sulfate (HS) concentrations in the cerebrospinal fluid (CSF), a biomarker for assessing effectiveness against CNS symptoms; these results were consistent with those obtained in pre-clinical studies.[3] Clinical studies have also demonstrated the positive effects of pabinafusp alfa on CNS symptoms.[4,5,6]

Pabinafusp alfa was approved in Japan by the Ministry of Health, Labour and Welfare and marketed since May 2021 under the brand name "IZCARGO™ I.V. Infusion 10mg" for the treatment of people with a rare lysosomal storage disorder.

About Fucosidosis

Fucosidosis is a lysosomal storage disorder that is inherited in an autosomal recessive manner. Mutations result in malfunction of a glycoprotein-metabolizing enzyme (α-L-fucosidase) which causes glycans and glycoproteins to accumulate throughout the body. Patients with fucosidosis display a variety of symptoms, including psychomotor symptoms, muscle hypotonia, visceromegaly, and skeletal abnormalities. Fucosidosis is classified into type I and type II according to the age of onset, and fewer than 120 cases have been reported worldwide, making it an ultra rare disease.

About Mucopolysaccharidosis Type II (Hunter Syndrome)

Mucopolysaccharidosis type II (MPS II, or Hunter syndrome) is an X-linked recessive lysosomal storage disorder caused by a deficiency of iduronate-2-sulfatase, an enzyme that breaks down complex carbohydrates called glycosaminoglycans (GAGs, also known as mucopolysaccharides) in the body. Hunter syndrome, which affects an estimated 2,000-3,000 individuals worldwide

(according to JCR research), gives rise to a wide range of somatic and neurological symptoms. The current standard of care for Hunter syndrome is enzyme replacement therapy. Central nervous system symptoms related to MPS II have been unmet medical needs so far.

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/.

References

1: Sonoda, et al. A blood-brain-barrier-penetrating anti-human transferrin receptor antibody fusion protein for neuronopathic mucopolysaccharidosis II. Mol. Ther. 2018; 26(5):1366-1374.

2: Morimoto, et al. Clearance of heparin sulfate in the brain prevents neurodegeneration and neurocognitive impairment in MPS II mice. Mol. Ther. 2021; 29(5): 1853-1861.

3: Okuyama, et al. Iduronate-2-sulfatase with Anti-human Transferrin Receptor Antibody for Neuropathic Mucopolysaccharidosis II: A Phase 1/2 Trial. Mol Ther. 2020; 27(2): 456-464.

4: Okuyama, et al. A Phase 2/3 Trial of Pabinafusp Alfa, IDS Fused with Anti-Human Transferrin Receptor Antibody, Targeting Neurodegeneration in MPS-II. Mol Ther. 2021; 29(2): 671-679.

5: Giugliani, et al. Iduronate-2-sulfatase fused with anti-human transferrin receptor antibody, pabinafusp alfa, for treatment of neuronopathic and non-neuronopathic mucopolysaccharidosis II: Report of a phase 2 trial in Brazil. Mol Ther. 2021; 29(7): 2378-2386.

6: Giugliani, et al. Enzyme Replacement Therapy with Pabinafusp Alfa for Neuronopathic Mucopolysaccharidosis II; an Integrated Analysis of Preclinical and Clinical Data. Int. J. Mol. Sci. 2021, Volume 22, Issue 20, 10938.

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