Life is Rare

May 28, 2026

JCR Pharmaceuticals Co., Ltd.

JCR Pharmaceuticals to Present at the 18th International Symposium on MPS and Related Lysosomal Diseases 2026

Hyogo, Japan – May 28, 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 new clinical data in a poster session at the 18th International Symposium on MPS and Related Lysosomal Diseases, being held June 4-7 in Florence, Italy. The poster presentation will demonstrate the potential benefits of the investigational therapy, JR-171(lepunafusp alfa), 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.

The details of the new clinical data presentation are listed below:

Title: *Three-Year safety and pharmacodynamics of lepunafusp alfa (JR-171) in patients with mucopolysaccharidosis type I (MPS-I): Results from a phase I/II trial and extension study*
Lead Author: *Paul Harmatz, M.D. (UCSF Benioff Children's Hospital, Oakland, CA)*
Presentation Number: *63*

In addition, researchers will also share the following encore presentations on JR-141 (pabinafusp alfa) in the treatment of MPS II that were disclosed at the 22nd Annual WORLD*Symposium*™ 2026 (February 2-6, 2026).

Title: *Sustained cognitive and adaptive behavior outcomes of long-term treatment with pabinafusp alfa in patients with severe or attenuated mucopolysaccharidosis type II*
Lead Author: *Roberto Giugliani, M.D., Ph.D. (Federal University of Rio Grande do Sul, Brazil)*
Presentation Number: *61*

Title: *Long-term somatic efficacy of pabinafusp alfa across a broad spectrum of age groups and phenotypes in patients with mucopolysaccharidosis type II*
Lead Author: *Ana Maria Martins, M.D., Ph.D. (Federal University of São Paulo)*
Presentation Number: *62*

Attendees who would like to receive more information about JCR Pharmaceuticals can visit JCR's on-site conference booth (#5).

The full program can be found on the International Symposium on MPS and Related Lysosomal Diseases website at https://mps2026.com/program. All posters will be available during the Networking Break and Poster Viewing sessions throughout the entire symposium.

About the International Symposium on MPS and Related Lysosomal Diseases

The International Symposium on MPS and Related Lysosomal Diseases brings together healthcare professionals, researchers, and industry leaders to accelerate progress in mucopolysaccharidoses (MPS) and related lysosomal storage disorders through collaborative innovation. We unite diverse perspectives to advance early diagnosis through cutting-edge technologies, develop revolutionary therapies, and ensure equitable global access to care. Our mission is to foster the next generation of rare disease advocates and professionals while creating sustainable partnerships that transform scientific breakthroughs into real-world improvements in patient outcomes worldwide. For more information, please visit https://mps2026.com/.

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 Mucopolysaccharidosis Type I (Hurler, Hurler-Scheie, Scheie Syndrome)

Mucopolysaccharidosis I (MPS I) is an autosomal recessive lysosomal storage disorder (LSD) caused by a deficiency of α-L-iduronidase, an enzyme that breaks down glycosaminoglycans (mucopolysaccharides) in the body. The current worldwide prevalence of MPS I is estimated to be approximately 3,000-4,000 (according to JCR internal research). MPS I gives rise to a wide range of somatic and neurological symptoms. A major limitation of current enzyme replacement therapy (ERT) is that it does not address central nervous system (CNS) symptoms because of the enzyme's inability cross the blood-brain barrier (BBB). MPS I is the only LSD in which hematopoietic stem cell transplantation (HSCT) is part of the standard of case for the severe form of the disease. Significant unmet medical need persists in all forms of MPS I.

About JR-171

JR-171 (lepunafusp alfa) is a recombinant fusion protein of an antibody against the human transferrin receptor and α-L-iduronidase, the enzyme that is missing or malfunctioning in patients with mucopolysaccharidosis type I (MPS I).[1,2] By crossing the blood brain-barrier (BBB) through transferrin receptor mediated transcytosis, it is expected to be effective against central nervous system (CNS) signs and symptoms of the disease thereby addressing a significant unmet need for the treatment of MPS I. JR-171 previously was granted Fast Track designation by the US Food and Drug Administration (FDA).

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 JR-141

JR-141 (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.[3,4]

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.[5] Clinical studies have also demonstrated the positive effects of pabinafusp alfa on CNS symptoms.[6,7,8]

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."

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

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