Life is Rare

February 5, 2026
JCR Pharmaceuticals Co., Ltd.

JCR Pharmaceuticals' Research Presentations at WORLD*Symposium*™ 2026 Showcase Data from Its Investigational Treatments for Lysosomal Storage Disorders

- Presentations Highlight Potential Benefits of Therapies Incorporating J-Brain Cargo®, JCR's Proprietary, Blood-Brain Barrier-Penetrating Technology -

Hyogo, Japan – February 5, 2026 – JCR Pharmaceuticals Co., Ltd. (TSE 4552; "JCR"), a global specialty biopharmaceutical company dedicated to developing therapies for rare and genetic diseases, today announced the presentation of four datasets demonstrating the potential benefits of its investigational therapies for lysosomal storage disorders (LSDs) at the 22nd Annual WORLD*Symposium*™ 2026. Researchers are presenting new data from two of its programs that apply the J-Brain Cargo® platform, a proprietary technology developed by JCR to deliver medicines across the blood-brain barrier (BBB), through four poster presentations this week.

"Lysosomal storage disorders are a group of rare diseases that have been notoriously difficult to treat due to the inability to deliver a therapy across the blood-brain barrier into the central nervous system. With our J-Brain Cargo® platform technology, we have the potential to address the progressive neurological symptoms associated with these devastating and life-limiting diseases, many of which have inadequate treatment options or no approved therapies available," said Shin Ashida, Chairman, President and CEO of JCR Pharmaceuticals. "The data presented at the WORLD*Symposium*™ demonstrate the growing body of safety and efficacy evidence of JR-141 in people with Hunter syndrome and highlight the potential of JR-471 for individuals affected by fucosidosis. We believe that our proprietary J-Brain Cargo® platform technology could be the foundation for therapies for lysosomal storage disorders. We wish to express our gratitude to the patients who have participated in our clinical programs, as well as their families, clinical investigators, and other collaborators who have supported us throughout the process."

The first dataset highlights pre-clinical data from the JR-471 clinical development program. JR-471 is an investigational BBB-penetrating α-L-fucosidase (rDNA origin) enzyme replacement therapy (ERT) that JCR is developing for the treatment of individuals affected by fucosidosis, in partnership with MEDIPAL HOLDINGS CORPORATION.

The other three datasets focus on the long-term efficacy and cognitive and adaptive behavioral effects of JR-141 (pabinafusp alfa) for mucopolysaccharidosis type II (MPS II, or Hunter syndrome). JR-141 is a recombinant iduronate-2-sulfatase (I2S) ERT that was approved in March 2021 by the Ministry of Health, Labour and Welfare (MHLW) in Japan, where it is marketed as IZCARGO™ for the treatment of people with MPS II.

JR-471 Dataset (Fucosidosis)

This poster presentation provides pre-clinical data from the JR-471 clinical development program investigating fucosidosis:

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 (Poster Number 246)
Presenter: Tomomi Masuda, Ph.D. (JCR Pharmaceuticals)

Researchers reported pre-clinical data on JR-471, a fusion protein of anti-human transferrin receptor 1 (TfR) antibody and human α-L-fucosidase (FUCA1) designed to cross the BBB by leveraging the mechanism of receptor-mediated transcytosis of transferrin. Researchers administered JR-471 intravenously once every week or once every other week for 26 weeks to human TfR knock-in and FUCA1 knockout (hTfR-KI/Fuca1-KO) mice, an animal model of fucosidosis. Researchers performed a rotarod test and an active avoidance test to evaluate the motor coordination and learning/memory function, respectively.

JR-471 treatment with either regimen reduced the Fuc-GlcNAc-Asn accumulated in central nervous system (CNS) tissues (e.g., the brain and cerebrospinal fluid), as well as in peripheral tissues by more than 95% at the maximum. Moreover, researchers found that there was a strong positive linear correlation between the Fuc-GlcNAc-Asn concentrations in the brain and cerebrospinal fluid (CSF). Concomitant with the effectiveness against substrate concentration in the brain, JR-471 treatment prevented a loss of Purkinje cells in the cerebellum. Researchers concluded that JR-471 may be a promising candidate for the treatment of fucosidosis due to its ability to cross the BBB, reduce accumulated substrate in central and peripheral organs, and limit the decline in motor coordination and learning/memory functions.

JR-141 Datasets (MPS II)

The following three poster presentations provide additional evidence and context for the use of JR-141 in the treatment of MPS II:

Sustained cognitive and adaptive behavior outcomes of long-term treatment with pabinafusp alfa in patients with severe or attenuated mucopolysaccharidosis type II (Poster Number 133)
Presenter: Roberto Giugliani, M.D., Ph.D. (Federal University of Rio Grande do Sul, Brazil)

In a longitudinal, pooled, *post hoc* analysis of patients with MPS II receiving pabinafusp alfa across five open-label trials, researchers reported on sustained cognitive and adaptive behavior outcomes of long-term treatment with pabinafusp alfa in patients with severe or attenuated MPS II. Sixty patients with MPS II were included (18 attenuated [mean age 20.8 years (range: 3–44); 15 received prior idursulfase ERT]; 42 severe [mean age 7.4 years (range: 0–23); 31 received prior idursulfase ERT]). Researchers determined neurocognition and adaptive behavior using the Bayley Scales of Infant and Toddler Development, third edition (BSID-III), the Kyoto Scale of Psychological Development (KSPD), and the Vineland Adaptive Behavior Scales, second edition (VABS-II).

Researchers observed cognitive improvement in patients with severe disease with baseline developmental quotient ≥55 (n=7) and patients with attenuated disease (BSID-III mean change from baseline to Week 260: +14.7 and +2.5, respectively). Adaptive behavior remained stable in severe patients; researchers observed improvements in patients with severe disease with baseline developmental quotient ≥55 and in patients with attenuated disease (VABS-II total raw mean change from baseline to Week 260: +152 and +33, respectively); they also noticed improvements across all VABS-II subdomains in these patients. Researchers concluded that long-term treatment with pabinafusp alfa was well tolerated and associated with stabilization or continued skill acquisition in many patients with severe or attenuated MPS II. These results suggest that, with timely initiation prior to the onset of irreversible neurodegeneration, treatment with pabinafusp alfa may provide a benefit to patients with MPS II.

Long-term somatic efficacy of pabinafusp alfa across a broad spectrum of age groups and phenotypes in patients with mucopolysaccharidosis type II (Poster Number 245)
Presenter: Ana Maria Martins, M.D., Ph.D. (Federal University of São Paulo)

In a longitudinal, pooled, *post hoc* analysis of patients with MPS II receiving pabinafusp alfa in open-label trials, researchers reported on the somatic effects of pabinafusp alfa in a heterogenous population of patients with MPS II who initiated treatment at different ages. Of 65 patients with MPS II, 42 had severe disease phenotype and 18 had attenuated disease phenotype.

In treatment-naïve patients (n=17), serum dermatan sulfate (DS) and serum heparan sulfate (HS) rapidly decreased following treatment with pabinafusp alfa (geometric mean change from baseline [gmCFB], Week 104: DS −65%; HS −77%), while levels were maintained in previously treated patients (n=47) through the end of follow-up. Researchers observed a similar pattern across patients treated at all ages and disease phenotype. In treatment-naïve patients, liver and spleen volumes (adjusted by body weight) decreased (gmCFB Week 104: −32% and −39%, respectively) and the left ventricular mass index (LVMI) stabilized by Week 52. In previously treated patients, there was a decreasing trend in liver and spleen volumes while LVMI remained stable (gmCFB, Week 104: −7%, −6%, and -3%, respectively). Researchers observed positive trends in somatic efficacy irrespective of prior ERT exposure and age at treatment initiation, with the greatest improvements observed in ERT treatment-naïve patients. They concluded that long-term treatment with pabinafusp alfa was well tolerated and provided positive somatic effects to a broad spectrum of patients with MPS II.

Infusion rate adjustment in enzyme replacement therapy with pabinafusp alfa for mucopolysaccharidosis II (Poster Number 310)
Presenter: Norio Sakai, M.D., Ph.D. (ISEIKAI International General Hospital, Osaka, Japan)

In this *post hoc*, retrospective pharmacodynamic analysis, researchers reviewed 260-week data from 27 Japanese patients with MPS II enrolled in a Phase II/III clinical trial and extension study of pabinafusp alfa to assess if a shorter infusion duration impacts the long-term efficacy and safety of ERT with pabinafusp alfa in patients with MPS II. Researchers evaluated pharmacodynamics using HS and DS levels in CSF, serum, and urine, along with neurocognitive development (KSPD), and liver and spleen volumes. Infusion duration was ≥3 hours (≤33mL/h) until Week 52; thereafter rates could be increased at physician discretion. Patients were retrospectively grouped by infusion speed as “fast” (n=18; ≥66% of pabinafusp alfa infusions during the extension period were administered at a rate of >33 mL/h (infusion duration predominantly <3 hours)) or “slow” (n=9; <66% of pabinafusp alfa infusions during the extension period were administered at a rate of >33 mL/h (infusion duration predominantly >3 hours)).

Shorter infusion times did not appear to correlate with increased infusion-associated reactions or other adverse events. Changes in HS and DS concentrations in CSF, serum and urine, as well as liver and spleen volumes, were similar regardless of infusion rate. These findings suggest that clinicians may safely consider shorter infusion durations when using pabinafusp alfa to treat MPS II to accommodate clinical circumstances or individual patient needs, potentially improving quality of life and treatment compliance in pediatric patients.

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 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."

Important Safety Information
INDICATION:
IZCARGO™ is indicated for the treatment of mucopolysaccharidosis type II (MPS II), which is also known as Hunter syndrome. IZCARGO™ is approved in Japan only.

CONTRAINDICATION:

IZCARGO™ is contraindicated in patients with a history of anaphylactic shock to its components.

WARNINGS AND PRECAUTIONS:

Warnings
Since serious anaphylaxis and shock may occur with use of IZCARGO™, adequate emergency measures should be made ready for execution before initiation of administration, and the patient should be closely monitored during and after the administration. If a serious infusion associated reaction (IAR) occurs, administration of IZCARGO™ should be discontinued, and appropriate actions should be taken.

When IZCARGO™ is administered to patients with severe respiratory failure or acute respiratory disease, an IAR may lead to acute exacerbation of symptoms. A patient's condition should be closely monitored, and appropriate actions should be taken as needed.

Precautions for Use
IZCARGO™ is a protein medicinal product and may cause anaphylactic shock, for which close monitoring is required. If any signs of anaphylaxis are noted, discontinue the infusion, and take appropriate actions. Considering the onset of such symptoms, emergency measures should be made ready for execution.

IZCARGO™ may cause IARs such as headache, chills, syncope, fatigue, dizziness, pyrexia, rash,

erythema, urticaria, or other symptoms. If an IAR occurs, reduce the rate or temporarily discontinue the infusion, and initiate appropriate drug treatment (e.g., corticosteroids, antihistamines, antipyretic analgesics, anti-inflammatory drugs) or emergency procedures (e.g., oxygen administration, securing of airway, adrenaline administration). Premedication with antihistamines, corticosteroids, etc., should be considered for the subsequent infusion of IZCARGO™.

ADVERSE REACTIONS:

The most commonly reported adverse reactions were pyrexia and urticaria.

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-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 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. There is no approved therapy available for this disease.

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/.

Cautionary Statement Regarding Forward-Looking Statements
This document contains forward-looking statements that are subject to known and unknown risks

and uncertainties, many of which are outside our control. Forward-looking statements often contain words such as "believe," "estimate," "anticipate," "intend," "plan," "will," "would," "target" and similar references to future periods. All forward-looking statements regarding our plans, outlook, strategy and future business, financial performance and financial condition are based on judgments derived from the information available to us at this time. Factors or events that could cause our actual results to be materially different from those expressed in our forward-looking statements include, but are not limited to, a deterioration of economic conditions, a change in the legal or governmental system, a delay in launching a new product, impact on competitors' pricing and product strategies, a decline in marketing capabilities relating to our products, manufacturing difficulties or delays, an infringement of our intellectual property rights, an adverse court decision in a significant lawsuit and regulatory actions.

This document involves information on pharmaceutical products (including those under development). However, it is not intended for advertising or providing medical advice. Furthermore, it is intended to provide information on our company and businesses and not to solicit investment in securities we issue.

Except as required by law, we assume no obligation to update these forward-looking statements publicly or to update the factors that could cause actual results to differ materially, even if new information becomes available in the future.

References

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