Life is Rare

October 14, 2026

JCR Pharmaceuticals Co., Ltd.

JCR Pharmaceuticals Receives Marketing Authorization in Kuwait for IZCARGO™, a Blood-Brain Barrier-Penetrating Enzyme Replacement Therapy for Hunter Syndrome

- This approval follows the recent marketing authorization for IZCARGO™ in the United Arab Emirates -

Hyogo, Japan – October 14, 2026 – JCR Pharmaceuticals Co., Ltd. (TSE 4552; "JCR"), a global specialty biopharmaceutical company dedicated to developing therapies for rare and genetic diseases, today announced that IZCARGO™ (pabinafusp alfa, or JR-141), a blood-brain barrier-penetrating enzyme replacement therapy (ERT) for Hunter syndrome developed using JCR's proprietary J-Brain Cargo® technology, received conditional marketing authorization in Kuwait.

Kuwait is the second market outside Japan where IZCARGO™ has received approval, following the United Arab Emirates in July 2026. IZCARGO™ was approved and launched in Japan in 2021 and is currently being evaluated in an ongoing global Phase III clinical trial.

JCR has been working to expand access to IZCARGO™ outside of Japan while advancing its global clinical development program. As part of this effort, JCR entered into a marketing and distribution agreement with Taiba Middle East FZ LLC ("Taiba") and pursued regulatory approval in Kuwait based on the product's marketing authorization in Japan. Under this agreement, JCR will also seek marketing authorization for IZCARGO™ in countries across the Middle East and North Africa (MENA) region, while Taiba will be responsible for sales and distribution in the relevant territories.

"The approval of IZCARGO™ in Kuwait is an important milestone for people living with Hunter syndrome, as they now have an option to manage this rare, life-limiting lysosomal storage disorder," said Hiroyuki Sonoda, Ph.D., President and Chief Scientific Officer of JCR Pharmaceuticals. "This marks the second marketing authorization of IZCARGO™ outside Japan, following its recent approval in the United Arab Emirates. We will work with Taiba to bring this therapy to more patients with Hunter syndrome and to continue expanding access to IZCARGO™ internationally for the patients who need it. Obtaining approval in Kuwait marks an important step in the overseas expansion of IZCARGO™."

"The approval of IZCARGO™ in Kuwait marks an important advancement in our efforts to broaden access to specialized treatments for rare diseases in the region," said Dr. Saif Al Hasani, Taiba Group CEO. "Building on the UAE approval, this milestone highlights the value of strong partnerships between regional healthcare organizations and global innovators in translating medical advancements into meaningful progress for patients. At Taiba, we remain focused on supporting sustainable access to innovative therapies and strengthening the region's capacity to address complex and underserved medical needs."

The impact of this announcement on JCR's consolidated financial results for this fiscal year (ending March 31, 2027) is expected to be minor.

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 Hunter syndrome, 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 Hunter Syndrome (Mucopolysaccharidosis Type II, or MPS II)

Hunter syndrome (mucopolysaccharidosis type II, or MPS II) 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 Pabinafusp Alfa

Pabinafusp alfa (JR-141) 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 pabinafusp alfa 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, the United Arab Emirates (UAE), and Kuwait.

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 Taiba Middle East FZ LLC
Taiba is a regional leader in the marketing, distribution, and commercialization of orphan and rare disease therapies across the Middle East and North Africa (MENA). With two decades of trusted collaboration with clinicians, hospitals, and global partners.

Taiba combines deep regional expertise with robust commercial execution—from regulatory approvals and patient access programs to strategic partnerships and long-term brand lifecycle management. The company connects healthcare systems with breakthrough treatments through early access and commercialization initiatives. Driven by a mission to close the treatment gap.

Taiba empowers medical institutions with cutting-edge solutions and exceptional service, ensuring patients with unmet medical needs gain timely and sustainable access to life-changing therapies. Please visit: http://www.taibarare.com/.

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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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.
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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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