

October 2, 2026

Italfarmaco S.P.A. | JCR Pharmaceuticals Co., Ltd.

Italfarmaco and JCR Pharmaceuticals Enter into Exclusive License Agreement for the Development and Potential Commercialization of Novel Investigational Therapy for Hunter Syndrome in the United States, Europe, and Latin America

- *JR-141 is a potentially transformative therapy designed to deliver enzymes to the brain and peripheral tissues to treat neuronopathic and somatic symptoms of Hunter syndrome through intravenous administration*
- *JCR is conducting a global Phase III clinical trial evaluating JR-141 in Hunter syndrome*
- *Italfarmaco will exclusively commercialize JR-141 in the United States, Europe, and Latin America upon regulatory approval*

Milan, Italy and Hyogo, Japan – October 2, 2026 – [Italfarmaco S.p.A.](#) (“Italfarmaco”) and [JCR Pharmaceuticals Co., Ltd.](#) (TSE 4552; “JCR”) today announced an exclusive license agreement to develop and commercialize JR-141 in the United States, Europe, and Latin America (“licensed territories”) upon regulatory approval. JR-141 is a blood-brain barrier (BBB) penetrating enzyme replacement therapy currently in Phase III ([NCT04573023](#)) evaluation as a treatment of Hunter syndrome (also known as mucopolysaccharidosis type II or MPS II). Approved and launched in Japan in 2021 as IZCARGO™ (INN: pabinafusp alfa), JCR and Italfarmaco seek to obtain regulatory approval for JR-141 from the U.S. Food and Drug Administration (FDA), the European Medicines Agency (EMA), the U.K. Medicines and Healthcare products Regulatory Agency (MHRA), and the Brazilian Health Regulatory Agency (ANVISA).

Following regulatory approvals by the FDA, EMA, MHRA and ANVISA, Italfarmaco will commercialize and distribute the product in the licensed territories, while JCR will retain responsibility for manufacturing JR-141 upon marketing authorizations. JCR will receive upfront, milestone and royalty payments, in addition to revenues associated with product supply.

JR-141 is a next-generation recombinant fusion protein of an antibody against the human transferrin receptor and iduronate-2-sulfatase, the enzyme that is missing or malfunctioning in individuals with Hunter syndrome. JR-141 was developed using J-Brain Cargo®, JCR's proprietary BBB-penetration technology. J-Brain Cargo® is engineered to transport the therapeutic enzyme across the BBB to directly reach the brain and address both the somatic and neuronopathic symptoms of the disease, which can lead to progressive cognitive decline.

This agreement expands the relationship between JCR and Italfarmaco, which entered into an exclusive license agreement in [December 2025](#) for the commercialization of givinostat in Japan, a treatment for Duchenne muscular dystrophy, together with a strategic partnership agreement for rare disease therapies.

“We are pleased to enter into this strategic agreement with Italfarmaco and collaborate with an ideal global commercialization partner as we work toward our goal of delivering JR-141 to people living with Hunter syndrome around the world,” said Hiroyuki Sonoda, Ph.D., President and Chief Scientific Officer of JCR Pharmaceuticals. “Italfarmaco has expertise in developing and commercializing therapies worldwide, and we look forward to working with Italfarmaco to make JR-141 available to patients outside Japan as soon as possible. This agreement extends our commitment to the partnership with Italfarmaco, developing therapies for rare and genetic diseases for patients around the globe.”

“This agreement marks an important milestone in the relationship between Italfarmaco and JCR Pharmaceuticals and reflects a shared commitment to advancing innovative therapies for people living with rare and genetic diseases,” said Francesco Di Marco, Chief Executive Officer of

Italfarmaco Group. “By bringing together JCR’s pioneering expertise in blood-brain barrier technologies and Italfarmaco’s global capabilities in rare disease development and commercialization, we are creating a strong foundation to accelerate innovation for underserved patient communities.”

“Building on the success of our existing collaboration in Duchenne muscular dystrophy, this agreement further strengthens our partnership and reinforces our shared ambition to be a leading force in the rare and genetic disease field,” said Antonio Nardi, Vice President and Head of Business & Portfolio Development of Italfarmaco. “Alongside our trusted partner, we look forward to advancing our mission of delivering transformative treatments to the patients and families who need them most.”

The upfront payment under this agreement has been incorporated into JCR’s consolidated earnings forecast for the fiscal year ending March 31, 2027.

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, which does not address the central nervous system-related symptoms of this lysosomal disease.

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 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.” It was also approved in the United Arab Emirates (UAE) in 2026.

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 and the United Arab Emirates (UAE).

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.

Prescribing information, indications, contraindications, warnings, and adverse reactions may differ by jurisdiction. Healthcare professionals should consult the locally approved prescribing information.

About Italfarmaco S.p.A.

Founded in 1938 in Milan, Italy, Italfarmaco is a private global pharmaceutical company that has led the successful development and approval of many pharmaceutical products around the world. The Italfarmaco group has operations in more than 90 countries through directly controlled or affiliated companies. The company is a leader in pharmaceutical research, product development, production and commercialization with proven success in multiple therapeutic areas including oncology, gynecology, neurology, cardiovascular disease and rare diseases. Italfarmaco's rare disease unit includes programs in Duchenne muscular dystrophy, Becker muscular dystrophy, amyotrophic lateral sclerosis and polycythemia vera.

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.
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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