

June 24, 2025

Translation

MEDIPAL HOLDINGS CORPORATION
JCR Pharmaceuticals Co., Ltd.**Notice of Orphan Drug Designation for JR-446
for Mucopolysaccharidosis Type IIIB by European Commission (EC)**

Tokyo and Hyogo, Japan – June 24 2025 – [MEDIPAL HOLDINGS CORPORATION](#) (TSE 7459, MEDIPAL) and [JCR Pharmaceuticals Co., Ltd.](#) (TSE 4552, JCR) today announced that the European Commission (EC) has granted orphan drug designation (ODD) to JR-446, an investigational drug for the treatment of mucopolysaccharidosis type IIIB (MPS IIIB or Sanfilippo syndrome type B), following the recent the U.S. Food and Drug Administration (FDA) designation.¹

MPS IIIB affects an estimated 500 to 1,000 individuals worldwide,² causing severe central nervous system (CNS) symptoms. Despite the dire need, there are currently no approved treatments available for this condition. JR-446, developed using JCR's proprietary J-Brain Cargo® technology, has shown promising non-clinical results in addressing the CNS symptoms of this challenging disorder, and it is currently being studied in a Phase I/II trial that is being conducted in Japan ([JR-446-101](#)) under a collaboration agreement between the two companies.

In September 2023, MEDIPAL and JCR entered into a licensing agreement in which MEDIPAL will commercialize JR-446 for MPS IIIB outside of Japan. In addition, MEDIPAL will support JCR in the clinical development of JR-446 in Japan, including the distribution of investigational drugs, disease awareness, and clinical trial advancement.³

With the ODD, JR-446 will be eligible for various incentives to encourage the development in the European Union (EU).

About Orphan Drug Designation in the European Union

The European Commission implements orphan designation drug for promoting new drug development for rare diseases in which the prevalence of the condition affects no more than five in 10,000 people in the European Union (EU). Designated drugs are granted market exclusivity for 10 years in the EU, as well as scientific guidance. Fee reductions are also available depending on the status of the sponsor and the type of service required.

About Mucopolysaccharidosis Type IIIB (Sanfilippo Syndrome Type B)

Mucopolysaccharidosis type IIIB, or Sanfilippo syndrome type B, is an autosomal recessive disease caused by pathogenic mutations in the *NAGLU* gene, encoding a lysosomal enzyme involved in the degradation of heparan sulfate. With the accumulation of heparan sulfate in the central nervous system in the brain, individuals with this condition present rapid neurological decline, including sleep disorders, loss of speech, and behavioral changes, which may significantly affect the quality of life of patients and their families.

About the J-Brain Cargo® Platform Technology

JCR Pharmaceuticals has developed a proprietary blood-brain barrier-penetrating technology J-Brain Cargo®, to bring biotherapeutics into the central nervous system. The first drug developed based on this technology is IZCARGO® (INN: pabinafusp alfa) and was approved in Japan for the treatment of a lysosomal storage disorder.

About MEDIPAL HOLDINGS CORPORATION

MEDIPAL is a holding company which controls, administers and supports the operating activities of companies in which it holds shares in the Prescription Pharmaceutical Wholesale Business; the Cosmetics, Daily Necessities and OTC Pharmaceutical Wholesale Business; and the Animal Health Products and Food Processing Raw Materials Wholesale and Related Business, and conducts business development for the MEDIPAL Group. For more information, visit <https://www.medipal.co.jp/english/>.

About JCR Pharmaceuticals Co., Ltd.

JCR is a global specialty pharmaceuticals company dedicated to advancing treatments for rare and genetic diseases. With nearly 50 years of expertise in Japan, JCR is expanding to the US, Europe, and Latin America. JCR's innovative therapies address conditions like growth disorder, MPS II, Fabry disease, acute graft-versus-host disease, and renal anemia. JCR is also developing treatments for rare diseases like MPS I, MPS II, MPS IIIA and B, and more. For more information, visit <https://www.jcrpharm.co.jp/en/site/en/>.

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

1. Reference: [Press release](#) on the orphan drug designation for JR-446 for Mucopolysaccharidosis Type IIIB by the U.S. FDA (May 7, 2025).
2. Based on data from JCR's own investigations, referring to the Ministry of Health, Labour and Welfare's public research.
3. Reference: [Press release](#) on the licensing agreement for JR-446 between MEDIPAL and JCR (September 28, 2023).

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