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Translation

MEDIPAL HOLDINGS CORPORATION  
JCR Pharmaceuticals Co., Ltd.**Notice of Orphan Drug Designation for JR-446  
for Mucopolysaccharidosis Type IIIB by the U.S. FDA**

Tokyo and Hyogo, Japan – May 7 2025 – [MEDIPAL HOLDINGS CORPORATION](#) (TSE 7459, MEDIPAL) and [JCR Pharmaceuticals Co., Ltd.](#) (TSE 4552, JCR) today announced that the U.S. Food and Drug Administration (FDA) 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).

MPS IIIB affects an estimated 500 to 1,000 individuals worldwide,<sup>1</sup> 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 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.

"We are extremely pleased that JR-446 has received orphan drug designation from the U.S. FDA," said Shuichi Watanabe, Representative Director, President and CEO of MEDIPAL. "We will continue the development of JR-446 in collaboration with JCR so that we can soon deliver this drug to the patients and their families who are desperately waiting, as there is currently no treatment available."

"This orphan drug designation is an important development step for the JR-446 clinical program," said Shin Ashida, President, Chairman and CEO of JCR Pharmaceuticals. "This orphan drug designation supports the potential of this therapy to address the significant unmet medical need of patients with MPS IIIB."

In September 2023, MEDIPAL and JCR entered into a licensing agreement in which MEDIPAL will commercialize JR-446 on MPS IIIB treatment 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.<sup>2</sup>

With the ODD, JR-446 will be eligible for various incentives to encourage the development in the U.S.

### **About Orphan Drug Designation in the U.S.**

The U.S. FDA's Office of Orphan Products Development grants orphan status to drugs being developed to treat, prevent, or diagnose a rare disease or condition affecting fewer than 200,000 people in the U.S. The designation provides significant incentives to promote the development of the drug including the potential for market exclusivity for seven years upon FDA approval, eligibility for tax credits for qualified clinical trials, and waiver of Prescription Drug User Fee Act Application fee.

### **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. The core values of reliability, confidence, and persistence drive JCR's mission to enhance global medical progress. 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. Based on data from JCR's own investigations, referring to the Ministry of Health, Labour and Welfare's public research.
2. Reference: [Press release](#) on the licensing agreement for JR-446 between MEDIPAL and JCR (September 28, 2023).

## **Contacts**

MEDIPAL HOLDINGS CORPORATION  
Public Relations Department  
TEL: (+81)-3-3517-5171

JCR Pharmaceuticals Co., Ltd.  
Corporate Communications  
TEL: (+81)-797-32-1995