

September 29, 2025

Translation

MEDIPAL HOLDINGS CORPORATION
JCR Pharmaceuticals Co., Ltd.

**Notice of Orphan Drug Designation for JR-446
for Mucopolysaccharidosis Type IIIB
by Ministry of Health, Labour and Welfare of Japan**

Tokyo and Hyogo, Japan – September 29, 2025 – [MEDIPAL HOLDINGS CORPORATION](#) (TSE 7459, MEDIPAL) and **[JCR Pharmaceuticals Co., Ltd.](#)** (TSE 4552, JCR) today announced that the Ministry of Health, Labour and Welfare of Japan has granted orphan drug designation to JR-446, an investigational drug for the treatment of mucopolysaccharidosis type IIIB (MPS IIIB or Sanfilippo syndrome type B).

JR-446 was granted orphan drug designation for the treatment of MPS IIIB by the U.S. Food and Drug Administration (FDA) in April 2025,¹ and by the European Commission (EC) in June 2025.²

MPS IIIB affects an estimated 500 to 1,000 individuals worldwide,³ 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.⁴

Both companies remain committed to developing a therapy for patients with MPS IIIB as swiftly as possible.

About Orphan Drug Designation in Japan

Under the orphan drug designation system, the Ministry of Health, Labour and Welfare of Japan takes special measures to support and promote research activities for the development of drugs for rare diseases. Drugs satisfying the following criteria may be designated as orphan drugs: they are targeting a condition with an incidence of fewer than 50,000 patients in Japan, or their intended

use is for the treatment of a designated intractable disease. The drugs should be indicated for the treatment of serious diseases including difficult-to-treat diseases for which there are high unmet medical needs, such as there is no approved medicinal product or higher efficacy, or safety is expected compared with an approved medicinal product. Moreover, the applicant for orphan drug designation needs to have enough resources and plans for development of its product as an orphan drug in Japan.

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 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 Pharmaceuticals Co., Ltd. is a global specialty pharmaceutical company that develops treatments that go beyond rare diseases to solve the world's most complex healthcare challenges. JCR continues to build upon our 50-year legacy in Japan while expanding our global footprint into 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://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

1. [Press release](#) on the orphan drug designation for JR-446 for Mucopolysaccharidosis Type IIIB by the U.S. FDA (May 7, 2025).
2. [Press release](#) on the orphan drug designation for JR-446 for Mucopolysaccharidosis Type IIIB by European Commission (EC) (June 24, 2025).
3. Based on data from JCR's own investigations, referring to the Ministry of Health, Labour and Welfare's public research.
4. [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
E-mail : ir-info@jp.jcrpharm.com