

July 1, 2024

Translation

MEDIPAL HOLDINGS CORPORATION
JCR Pharmaceuticals Co., Ltd.

**MEDIPAL and JCR Announce Completion of Clinical Trial Notification
Process in Japan for Phase I/II Study of JR-446
for Mucopolysaccharidosis Type IIIB**

July 1, 2024 -- MEDIPAL HOLDINGS CORPORATION (TSE 7459 “MEDIPAL”) and JCR Pharmaceuticals Co., Ltd. (TSE 4552 “JCR”) today announced the completion of the regulatory review by the Pharmaceuticals and Medical Devices Agency (PMDA) for the clinical trial notification for the phase I/II study of JR-446, a blood-brain barrier-penetrating α -N-acetylglucosaminidase, for the treatment of mucopolysaccharidosis type IIIB (MPS IIIB; Sanfilippo syndrome type B), a devastating and ultra-rare lysosomal storage disorder.

MPS IIIB affects an estimated 500 to 1,000 individuals worldwide¹, causing severe central nervous system 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 preclinical results in addressing the symptoms of this challenging disorder.

In September 2023, MEDIPAL and JCR secured a license to commercialize JR-446 overseas and partnered to develop and commercialize it in Japan, targeting MPS IIIB². With the regulatory review now complete, we plan to start the clinical trial in Japan in the first half of FY2024 (April to September).

This collaboration highlights the commitment of MEDIPAL and JCR to pioneer treatments for ultra-rare diseases. By advancing therapies like JR-446, we aim to bring hope to patients and their families, while enhancing corporate value and contributing to a society where everyone can live with physical and mental well-being.

¹ Based on data from the Ministry of Health, Labour and Welfare’s public research and JCR’s own investigations.

² Reference: [Press release](#) on the licensing agreement for JR-446 between MEDIPAL and JCR (September 28, 2023).

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 is a global specialty pharmaceuticals company dedicated to advancing treatments for rare and genetic diseases. With nearly 50 years of expertise in Japan, we are expanding to the US, Europe, and Latin America. Our innovative therapies address conditions like growth disorder, MPS II, Fabry disease, acute graft-versus-host disease, and renal anemia. We are also developing treatments for rare diseases like MPS I, MPS II, MPS IIIA and B, and more. Our core values of reliability, confidence, and persistence drive our mission to enhance global medical progress.

For more information, visit <https://www.jcrpharm.co.jp/en/site/en/>.

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