MEDIPAL HOLDINGS CORPORATION

News Release

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MEDIPAL HOLDINGS CORPORATION
JCR Pharmaceuticals Co., Ltd.

MEDIPAL HOLDINGS CORPORATION and JCR Pharmaceuticals Dose First Patient in Global Phase I/II Trial of JR-446 for Mucopolysaccharidosis Type IIIB

- The study aims to evaluate the safety, tolerability, and preliminary efficacy of JR-446 for the treatment of people with MPS IIIB, for which there is no approved therapy -

Tokyo and Hyogo, Japan – September 11, 2026 – MEDIPAL HOLDINGS CORPORATION (TSE 7459, MEDIPAL) and JCR Pharmaceuticals Co., Ltd. (TSE 4552, JCR) announced today that the first participant has been dosed in the global Phase I/II trial of JR-446 for mucopolysaccharidosis type IIIB (Sanfilippo syndrome type B; "MPS IIIB"). JR-446 is a blood-brain barrier-penetrating α-N-acetylglucosaminidase developed by JCR using its proprietary J-Brain Cargo® technology. MEDIPAL holds commercialization rights for JR-446 outside Japan.

MPS IIIB is a lysosomal storage disorder that causes severe central nervous system impairment. This ultra-rare disease is estimated to affect approximately 500 to 1,000 people worldwide,[1] and there is currently no approved treatment.

"Dosing the first patient in this Phase I/II clinical trial is an important milestone for the MPS IIIB community, as there is no approved treatment for this devastating and life limiting lysosomal storage disorder that impacts patients' neurocognitive development, independence, and quality of life," said Dr. Irene Chang, Principal Investigator, Associate Professor of Pediatrics, Section of Biochemical Genetics, Division of Medical Genetics, at the University of California, San Francisco (UCSF). "Patients and families affected by this condition continue to face significant unmet medical needs, and we are encouraged to take this next step in evaluating the potential of this investigational therapy. We are grateful to the patients, caregivers, and study teams whose commitment made this milestone possible, and we are hopeful it may bring meaningful improvements in the quality of life for individuals living with MPS IIIB and their families."

In September 2023, MEDIPAL and JCR entered into a licensing agreement for the commercialization of JR-446 for MPS IIIB outside Japan and a co-development and commercialization agreement in Japan.[2] Under this agreement, MEDIPAL holds exclusive worldwide commercialization rights, excluding Japan, with the right to sublicense. On behalf of MEDIPAL, JCR is leading the global Phase I/II trial and development activities as the sponsor.

The global, multicenter, open-label trial is being conducted in patients with MPS IIIB under six years of age. Further information is available at ClinicalTrials.gov under identifier NCT07640984.

Nonclinical studies of JR-446 have demonstrated effects on symptoms associated with MPS IIIB. A separate Phase I/II trial, JR-446-101, is ongoing in Japan under the companies' co-development agreement. In 2025, JR-446 received orphan drug designation in the United States, the European Union, and Japan.

MEDIPAL and JCR remain committed to advancing JR-446 with the goal of bringing a new treatment option to people with MPS IIIB as quickly as possible.

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 (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 and the United Arab Emirates for the treatment of a lysosomal storage disorder (LSD). With J-Brain Cargo®, JCR and MEDIPAL seek to address the unresolved clinical challenges of LSDs by delivering the enzyme to both the body and the brain.

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 its 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. Based on data from JCR's own investigations, referring to the Ministry of Health, Labour and Welfare's public research.
2. Press release on the licensing agreement for JR-446 between MEDIPAL and JCR (September 28, 2023).

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