

August 29, 2025

Translation

MEDIPAL HOLDINGS CORPORATION  
JCR Pharmaceuticals Co., Ltd.**MEDIPAL HOLDINGS and JCR Pharmaceuticals Sign Global Licensing and Japan Co-Development Agreement for JR-479, a Novel Therapy for GM2 Gangliosidosis**

Tokyo and Hyogo, Japan – August 29, 2025 – [MEDIPAL HOLDINGS CORPORATION](#) (TSE 7459, MEDIPAL) and [JCR Pharmaceuticals Co., Ltd.](#) (TSE 4552, JCR) today announced that the two companies have signed an exclusive global licensing deal and a co-development and commercialization partnership in Japan for JR-479, an investigational therapy for GM2 gangliosidosis, an ultra-rare lysosomal storage disorder.

GM2 gangliosidoses are a group of rare, inherited disorders caused by genetic mutations that prevent the normal breakdown of GM2 gangliosides in lysosomes, leading to their buildup, especially in neurons. Tay-Sachs disease and Sandhoff disease are the two most common types of GM2 gangliosidosis depending on the specific mutation. GM2 gangliosidosis affects an estimated one in 300,000 people worldwide and is considered an ultra-rare, life-threatening disorder for which no approved treatments currently exist.

JR-479 is a novel drug candidate developed with JCR's proprietary J-Brain Cargo® platform, clinically validated through IZCARGO® for I.V. infusion 10mg, which gained approval in Japan in 2021 for the treatment of a lysosomal storage disorder. In preclinical studies,<sup>1</sup> JR-479 was delivered throughout the body, including to the central nervous system, reducing disease-related substrates and extending survival in animal models.

Under the global licensing deal, MEDIPAL will have exclusive worldwide rights (excluding Japan) to develop, manufacture, and commercialize JR-479, with sub-licensing rights. In Japan, through the co-development partnership, MEDIPAL will handle clinical trial logistics, support disease awareness, and assist with operations, while JCR leads commercialization and records domestic revenue. JCR will also receive an upfront payment, and royalties on overseas sales.

This agreement adds JR-479 to the strategic collaboration between MEDIPAL and JCR, launched in October 2022, to develop treatments for ultra-rare diseases. It follows JR-471 for fucosidosis<sup>2</sup> and JR-446 for mucopolysaccharidosis type IIIB,<sup>3</sup> making JR-479 the third pipeline in the partnership.

Both companies have already factored the financial impact of this agreement into their consolidated forecasts for the fiscal year ending March 31, 2026.

### **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 rare lysosomal storage disorder.

### **About lysosomal storage diseases**

Lysosomal storage diseases are genetic disorders in which certain enzymes or proteins that remove unwanted substances from cells do not work as they should. When that happens, those substances build up inside the lysosomes and damage cells and tissues. The symptoms differ depending on what builds up, and many of these conditions also affect the central nervous system.

### **About GM2 Gangliosidosis (Tay-Sachs, Sandhoff disease)**

GM2 gangliosidosis is a group of rare, inherited lysosomal storage disorders caused by deficiency of  $\beta$ -hexosaminidase A (Hex A), an enzyme consisting of  $\alpha$  and  $\beta$  subunits. Depending on whether the  $\alpha$ - or  $\beta$ - subunit is deficient, GM2 gangliosidosis is classified as Tay-Sachs or Sandhoff disease, respectively. Deficiency of Hex A results in the progressive accumulation of GM2 gangliosides and other glycolipids, which leads to severe neurodegeneration. In severe cases, nearly half of affected children do not survive past the age of three.<sup>4</sup> Currently, there are no approved treatments, highlighting the urgent need for new therapies.

### **About MEDIPAL HOLDINGS CORPORATION**

MEDIPAL HOLDINGS CORPORATION 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 dedicated 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 research presentations at WORLDSymposium™ 2025 (February 5, 2025).
2. [Press release](#) on the exclusive negotiation rights for global commercialization targeting four ultra-rare diseases and the licensing contract for the fucosidosis therapeutic between MEDIPAL and JCR (October 31, 2022).
3. [Press release](#) on the licensing agreement for JR-446 between MEDIPAL and JCR (September 28, 2023).
4. Bley AE, et al. Natural history of infantile G(M2) gangliosidosis. *Pediatrics*. 2011;128(5): e1233-1241.

## **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](mailto:ir-info@jp.jcrpharm.com)