BioSpectrum
the business of Bio & Health Sciences
ASIA EDITION

Japan's Medipal and JCR Pharma to co-develop novel therapy for rare lysosomal storage disorder

September 2, 2025 | News

JR-479 is a novel drug candidate developed with JCR's proprietary J-Brain Cargo platform



Medipal Holdings Corporation and JCR Pharmaceuticals Co., have signed an exclusive global licensing deal and a co-development and commercialisation 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.

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, 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 commercialise 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 commercialisation and records domestic revenue. JCR will also receive an upfront payment, and royalties on overseas sales.