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Japanese firms JCR Pharmaceuticals and Modalis Therapeutics move forward with development of novel gene therapy

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Opening the door to innovative therapeutic possibilities that could make a meaningful difference

JCR Pharmaceuticals Co. and Modalis Therapeutics Corporation have validated the initial proof of concept in a joint research programme for the development of a novel gene therapy for a central nervous system (CNS) disease. Due to the success of the partnership thus far, Modalis and JCR have agreed to proceed to the next phase of their research by entering into a new joint research agreement.

The purpose of this joint agreement is to conduct pre-clinical studies for the development of a new gene therapy for the undisclosed CNS disease by applying J-Brain Cargo, JCR's proprietary technology that is able to cross the blood-brain barrier (BBB), and a gene therapy payload based on CRISPR-GNDM (Guide Nucleotide-Directed Modulation), Modalis' proprietary epigenome modulation technology which does not cleave or alter DNA sequences.

The two companies began a joint research collaboration in December of 2023 to evaluate the drug delivery technology of the gene therapy to the CNS. As a result of this research, the initial proof of concept has been validated. The next phase of the agreement is to jointly develop a novel and innovative gene therapy to provide patients with improved efficacy, safety, and less burden via intravenous injection (IV) in a minimally invasive and efficient manner.

According to Haruhiko Morita, CEO of Modalis, "We believe that CRISPR-GNDM has huge potential in the field of CNS diseases. So, the combination with J-Brain Cargo technology could be a very significant breakthrough to maximise the potential and value of our proprietary epigenome editing technology (CRISPR-GNDM) in CNS diseases."

This announcement is expected to have a minor impact on both companies' consolidated financial results for this fiscal year (JCR: ending March 2025, Modalis: ending December 2025).