News Release

January 6, 2025

JCR Pharmaceuticals Co., Ltd.
Modalis Therapeutics Corporation

JCR Pharmaceuticals and Modalis Therapeutics Announce Transition to the Next Phase of Joint Research Agreement for Development of Novel Gene Therapy

Hyogo, Japan and Tokyo & Waltham, MA – January 6, 2025 – JCR Pharmaceuticals Co., Ltd. (TSE 4552; "JCR") and Modalis Therapeutics Corporation (TSE 4883; "Modalis") today announced that they have validated the initial proof of concept in a joint research program 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.

"I am pleased to share the progress we've achieved in our collaboration with Modalis," said Hiroyuki Sonoda, Ph.D., Director, Senior Managing Executive Officer, and Executive Director, Research Division at JCR. "By combining our proprietary J-Brain Cargo® technology for BBB penetration with Modalis' CRISPR-GNDM® platform for epigenome editing, we are opening the door to innovative therapeutic possibilities that could make a meaningful difference."

"We are very happy to move the joint research collaboration with JCR to the next phase," said Haruhiko Morita, CEO of Modalis. "This is the result of hard work on both companies and an important strategic step for Modalis' research activities. As a pioneer in this technology, we have demonstrated promising long-term drug efficacy in mouse disease model studies, including demonstration of target engagement and safety in non-human primates, exhibiting strong biodistribution for our lead program in neuromuscular disorders. 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 maximize 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).

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/.

About Modalis Therapeutics Corporation

Modalis was founded in 2016 and conducts its research and development in Massachusetts, USA. Modalis is a forerunner in the field of epigenetic medicine. Modalis develops therapeutics for patients suffering from serious genetic disorders such as neuromuscular diseases, CNS diseases, and cardiomyopathies. Modalis'proprietary CRISPR-GNDM® technology is capable of specifically modulating the expression of disease-relevant genes without introducing double-strand DNA breaks. For more information, visit https://www.modalistx.com/en/.

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.

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