



December 20, 2023
JCR Pharmaceuticals Co., Ltd.

Translation

JCR Announces Research Collaboration, Option and License Agreement with Alexion on Discovery of Oligonucleotide Therapeutics Using J-Brain Cargo® Technology

New collaboration aims to create innovative oligonucleotide therapeutics that enable target delivery to certain tissues and organs of the human body in rare diseases

Hyogo, Japan, Dec. 20, 2023 -- [JCR Pharmaceuticals Co., Ltd.](#) (TSE 4552; Chairman and President: Shin Ashida: “JCR”) announces that it has signed a Research Collaboration, Option and License Agreement with Alexion, AstraZeneca Rare Disease (“Alexion”) for the development of novel oligonucleotide therapeutics to support targeted delivery to certain tissues or organs using J-Brain Cargo®, JCR’s proprietary blood-brain barrier (BBB)-penetrating technology.

Under the terms of the agreement, JCR and Alexion will research and develop oligonucleotide therapeutics using J-Brain Cargo® Technology. As part of the collaboration, Alexion may nominate up to three targets in rare disease indications for pre-clinical development using the platform. Following pre-clinical development, Alexion will have the option to develop and commercialize the potential new drug candidates.

The collaboration is the second agreement between JCR and Alexion in 2023, following [a March announcement](#) regarding a research collaboration, option and license agreement to develop therapeutic protein candidates using J-Brain Cargo® for neurodegenerative disease.

JCR will receive an upfront payment from Alexion and has the right to receive certain research, development and commercial milestones and royalties.

“I am excited that Alexion and JCR can further extend their collaboration into nucleic acid medicines”, said Shin Ashida, Chairman and President of JCR Pharmaceuticals. “The agreement is another testament of the versatile applicability of the J-Brain Cargo® Technology to different modalities and of JCR being a partner of choice for the development of biotherapeutics to support targeted delivery to certain tissues or organs”.

J-Brain Cargo® technology is JCR’s proprietary drug delivery system that efficiently delivers drugs to target tissues, including the central nervous system, through receptor-mediated transcytosis. It is applicable to various modalities including enzymes, oligonucleotides, lipid nanoparticles, gene and cell therapy, peptides, decoy receptors and antibodies.

There is minor impact on JCR's consolidated business results for this fiscal year ending on March 31, 2024 related to the matter.

About the J-Brain Cargo® Platform Technology

J-Brain Cargo® is a proprietary blood-brain barrier (BBB)-penetrating technology to bring biotherapeutics into the central nervous system (CNS). The first drug developed based on this technology and approved in Japan for a lysosomal storage disorder is IZGARGO® (INN: pabinafusp alfa). Based on the same platform technology, JR-141 (INN: pabinafusp alfa), JR-171 (INN: lepunafusp alfa) and JR-441 are advancing into global clinical trials. JCR intends to start clinical trials on four additional programs from its LSD pipeline by FY2028.

About JCR Pharmaceuticals Co., Ltd.

JCR Pharmaceuticals Co., Ltd. (TSE 4552) is a global specialty pharmaceuticals company that is redefining expectations and expanding possibilities for people with rare and genetic diseases worldwide. We continue to build upon our 48-year legacy in Japan while expanding our global footprint into the US, Europe, and Latin America. We improve patients' lives by applying our scientific expertise and unique technologies to research, develop, and deliver next-generation therapies. Our approved products in Japan include therapies for the treatment of growth disorder, Fabry disease, acute graft-versus host disease, and renal anemia. Our investigational products in development worldwide are aimed at treating rare diseases including MPS I (Hurler, Hurler-Scheie and Scheie syndrome), MPS II (Hunter syndrome), MPS III A and B (Sanfilippo syndrome type A and B), and more. JCR strives to expand the possibilities for patients while accelerating medical advancement at a global level. Our core values – reliability, confidence, and persistence – benefit all our stakeholders, including employees, partners, and patients. Together we soar. For more information, please visit <https://www.jcrpharm.co.jp/en/site/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.

Contact:

Investors & Media:

JCR Pharmaceuticals Co., Ltd.

Corporate Communications

ir-info@jp.jcrpharm.com

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