



March 30, 2023
JCR Pharmaceuticals Co., Ltd.

Translation

JCR Pharmaceuticals Announces Research Collaboration, Option and License Agreement with Alexion to Develop a Therapy Using J-Brain Cargo® for Neurodegenerative Disease

Hyogo, Japan, Mar. 30, 2023 – [JCR Pharmaceuticals Co., Ltd.](#) (TSE 4552; Chairman and President: Shin Ashida; “JCR”) today announced that JCR has signed Research Collaboration, Option and License Agreement with Alexion, AstraZeneca Rare Disease (“Alexion”) to develop an undisclosed initial therapeutic molecule that applies JCR’s proprietary J-Brain Cargo®, blood-brain barrier (“BBB”) penetration technology, for the treatment of a neurodegenerative disease.

J-Brain Cargo®, which is a JCR proprietary technology, allows the delivery of biotherapeutics into the central nervous system (“CNS”) via a mechanism called receptor-mediated transcytosis. The first drug approved in Japan using the J-Brain Cargo® technology is IZCARGO® for the treatment of mucopolysaccharidosis II, a lysosomal storage disease with significant central nervous signs and symptoms. Since the platform technology has broad applicability to many therapeutic effectors, it has the potential to treat multiple CNS indications. The agreement with Alexion is the first international partnership to apply the J-Brain Cargo® technology for the treatment of a neurodegenerative disease.

Under the terms of the agreement, the two companies will begin research and development of new treatments using the J-Brain Cargo® Technology in combination with an undisclosed effector molecule. During the research period, Alexion also has the option to nominate additional therapeutic protein candidates in undisclosed indications. Following pre-clinical development, Alexion may exercise its option to further develop and commercialize new drug candidates under this collaboration. JCR will receive an immediate upfront payment from Alexion and has the right to receive certain research, development and commercial milestones and royalties upon the achievement of certain milestone events, including on post approval net sales.

“The collaboration with Alexion is a milestone in the recognition of the J-Brain Cargo® Technology as it is for the first time applied for the treatment of a neurodegenerative disease” said Shin Ashida, President and Chairman of JCR, further adding “we are very pleased to collaborate with Alexion as a partner in this effort. We are confident that this new partnership will lead to new therapies for the treatment of neurodegenerative disease an area with high unmet need and desperately needed therapeutic innovation.”

The impact of this Research Collaboration, Option and License Agreement on the consolidated business results for the fiscal year ending March 31, 2023 has been incorporated into the business forecast for the current fiscal year.

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. 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.

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