



March 19, 2024
JCR Pharmaceuticals Co., Ltd.

Translation

JCR Pharmaceuticals Announces Achievement of Milestone Using J-Brain Cargo® Technology for Neurodegenerative Disease in Research Collaboration with Alexion

Hyogo, Japan, Mar. 19, 2024 – [JCR Pharmaceuticals Co., Ltd.](#) (TSE 4552; Chairman and President: Shin Ashida; “JCR”) today announced that it has achieved the first research milestone criterion in a collaboration with Alexion, AstraZeneca Rare Disease (“Alexion”) to develop an undisclosed initial therapeutic molecule that applies JCR’s proprietary J-Brain Cargo®, blood-brain barrier penetration technology, for the treatment of a neurodegenerative disease. (Related release is [here](#)). The achievement of a predefined research milestone triggers a milestone payment to JCR.

JCR recently strengthened its collaboration with Alexion to address unmet needs by initiating a second research collaboration in December 2023 to apply the same technology to oligonucleotide therapeutics. (Related release is [here](#)).

The milestone payment has a minor impact on JCR’s consolidated financial results for this fiscal year ending on March 31, 2024.

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. The first drug developed based on this technology and approved in Japan for a lysosomal storage disorder (LSD) is IZGARGO® (INN: pabinafusp alfa) for the treatment of Mucopolysaccharidosis(MPS) type II (Hunter Syndrome). Based on the same platform technology, JR-141(INN: pabinafusp alfa), JR-171 for the treatment of MPS I (INN: lepunafusp alfa) and JR-441 for the treatment of MPS IIIA have advanced into global clinical trials. JCR intends to start clinical trials with 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 49-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, MPS II, 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 progress 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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