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JCR Pharmaceuticals Co., Ltd.

JCR Pharmaceuticals and Alexion Achieve Milestone in Collaborative Neurodegenerative Disease Program

Hyogo, Japan – September 19, 2025 – [JCR Pharmaceuticals Co., Ltd.](#) (TSE 4552; “JCR”), a global specialty biopharmaceutical company dedicated to developing therapies for rare and genetic diseases, has achieved a key research milestone in [collaboration with Alexion](#), AstraZeneca Rare Disease (“Alexion”), focused on applying JCR’s proprietary J-Brain Cargo® technology to develop a therapeutic protein candidate for a neurodegenerative disease. The achievement triggers a milestone payment from Alexion to JCR.

JCR and Alexion are advancing additional initiatives aimed at addressing areas of significant unmet medical need, including a [December 2023 agreement](#) to explore oligonucleotide therapeutics with J-Brain Cargo® and a [July 2025 license agreement](#) to develop genomic medicines utilizing JCR’s JUST-AAV platform.

This milestone and the related payment have already been factored into JCR’s earnings forecast for the fiscal year ending March 31, 2026.

About the J-Brain Cargo® Platform Technology

JCR Pharmaceuticals has developed a proprietary blood-brain barrier-penetrating technology, J-Brain Cargo®, to bring biotherapeutics into the central nervous system. The first drug developed based on this technology and approved in Japan for the treatment a lysosomal storage disorder is IZCARGO® (INN: pabinafusp alfa).

About the JUST-AAV Platform Technology

JUST-AAV is a proprietary gene therapy platform technology that utilizes modified adeno-associated virus (AAV) vectors. The technology entails insertion of miniaturized antibodies against receptors on selected tissues, organs or the blood-brain barrier onto the capsid surface, enhancing targeted delivery to those tissues and organs. Further capsid modifications minimize off-target effects and improve safety. The name is derived from “**J**CR” “**U**ltimate destination of organ” “**S**afeguarding against off-target delivery” and “**T**ransformative technology” reflecting its potential for broad application across various diseases.

About JCR Pharmaceuticals Co., Ltd.

JCR Pharmaceuticals Co., Ltd. (TSE 4552) is a global specialty pharmaceutical company that develops treatments that go beyond rare diseases to solve the world’s most complex healthcare challenges. We continue to build upon our 50-year legacy in Japan while expanding our global footprint into the U.S., 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, MPS II (Hunter syndrome), 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, MPS IIIA and B (Sanfilippo syndrome type A and B), and more. Our core values – Putting people first, Forging our own path,

Always advancing, and Committed to excellence – mean that the work we do benefits all our stakeholders, including partners, patients and employees. We strive to expand the possibilities for patients while accelerating medical advancement at a global level. For more information, please visit JCR's global website: <https://jcrpharm.com/>.

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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