



July 8, 2025

JCR Pharmaceuticals Co., Ltd.

## **JCR Pharmaceuticals Enters License Agreement with Alexion for Proprietary JUST-AAV Capsids to be Used in the Development of Genomic Medicines**

**Hyogo, Japan – July 8, 2025 – [JCR Pharmaceuticals Co., Ltd.](#)** (TSE 4552; “JCR”) today announced that it has entered into a license agreement with Alexion, AstraZeneca Rare Disease (“Alexion”), for JCR’s new, proprietary JUST-AAV capsids to develop genomic medicines.

JUST-AAV encompasses a range of vector types optimized for various target tissues—including liver-sparing, muscle-targeting, and brain-targeting variants—to expand the potential of AAV-based gene therapy.

Under the terms of the agreement, Alexion may use the licensed capsids, which are part of the JUST-AAV platform, in up to five of Alexion's genomic medicines programs. JCR will receive an upfront payment from Alexion. JCR is eligible to receive milestone payments of up to USD 225 million related to research and development, and up to USD 600 million related to sales, for a total of up to USD 825 million (approximately JPY 120 billion converted at the exchange rate of JPY 145 to USD), which reflects the aggregate milestones if all five targets are exercised. In addition, JCR is entitled to receive tiered royalties based on net sales.

This agreement marks the third partnership between JCR and Alexion, following research collaborations involving J-Brain Cargo® technology—the [first](#) announced in March 2023 for neurodegenerative disease and [the second](#) announced in December 2023 for the discovery of oligonucleotide therapeutics.

“I am very pleased to announce the signing of this license agreement for our proprietary AAV capsid platform, JUST-AAV” said Shin Ashida, Chairman, President and CEO of JCR. “I believe JUST-AAV has the potential to make a real difference in the development of gene therapies for rare diseases, and I look forward to its application across a broader range of conditions. JCR values its partnership with Alexion, and we are pleased to build on the collaboration we have developed over the years.”

The impact of this agreement on JCR’s consolidated financial results for the fiscal year ending March 31, 2026, has already been factored into the current earnings forecast.

### **About JUST-AAV**

JUST-AAV is a proprietary 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 “JCR” “**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 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 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 employees, partners, and patients. We strive to expand the possibilities for patients while accelerating medical advancement at a global level. For more information, please visit the 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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