



July 18, 2024  
JCR Pharmaceuticals Co., Ltd.

Translation

## **JCR Pharmaceuticals Highlights Innovative Gene Therapy Research at the 7th International Forum of Lysosomal Disorders**

July 18, 2024 -- [JCR Pharmaceuticals Co., Ltd.](https://www.jcr-pharm.com) (TSE 4552: Chairman and President Shin Ashida, "JCR") announced presentations highlighting its latest advancements in gene therapy research at the 7th International Forum of Lysosomal Disorders, held in Tokyo on July 12-13, 2024.

At the forefront of this groundbreaking research is JCR's proprietary J-Brain Cargo® technology, modified to the surface of an adeno-associated virus (AAV) for enhanced central nervous system drug delivery. Non-clinical trials on mice and monkeys have showcased the efficient delivery of the AAV vector to the brain. Unlike traditional AAV vectors that primarily accumulate in the liver, our new AAV vector utilizing J-Brain Cargo® showed significantly reduced liver accumulation, effectively targeting the brain. This breakthrough points towards a potential therapy that mitigates the risk of liver toxicity commonly associated with AAV treatments.

"The non-clinical data we have presented demonstrates the remarkable potential of our proprietary J-Brain Cargo® technology for gene therapy," said Shin Ashida, Chairman and President of JCR. "By achieving efficient vector delivery to the brain and reducing liver-related side effects, we believe our technology can become a new platform for developing safer and more effective treatments."

### **About the International Forum of Lysosomal Disorders**

Established in 2007, the forum hosts a platform for global leaders in genetic disease research to explore and discuss key areas of lysosomal storage disorders. It aims to foster the development of internationally acclaimed researchers in Japan. (<https://ifld2024.jp/>)

### **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 of MPS II (mucopolysaccharidosis type II) is IZCARGO® (INN: pabinafusp alfa).

### **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, 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. 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. 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:

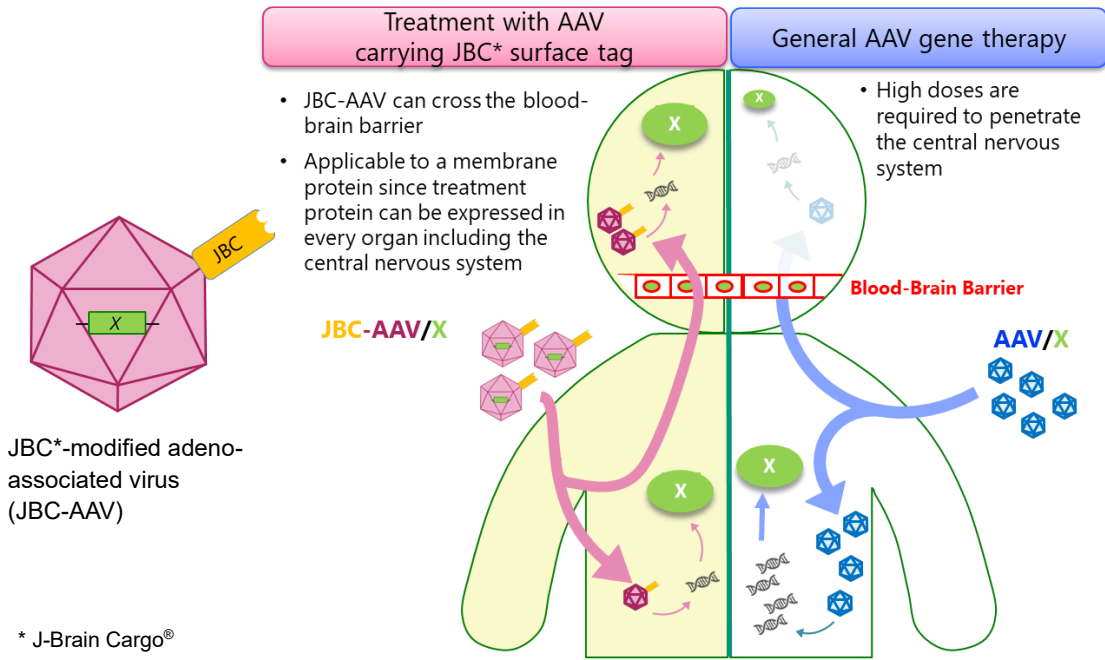
Investors & Media:

JCR Pharmaceuticals Co., Ltd.

Corporate Communications

[ir-info@jp.jcrpharm.com](mailto:ir-info@jp.jcrpharm.com)

Reference:



###