



March 28, 2022
JCR Pharmaceuticals Co., Ltd.

Translation

JCR Pharmaceuticals Announces Exclusive License and Collaboration Agreement with Takeda to Develop Gene Therapies Using J-Brain Cargo® Technology for Lysosomal Storage Disorders

Hyogo, Japan, March 28, 2022 -- [JCR Pharmaceuticals Co., Ltd. \(TSE:4552\)](https://www.jcr-pharm.co.jp/en) (“JCR”) announced today that it has entered into a research and development collaboration and exclusive license agreement with Takeda to develop gene therapies that apply JCR’s proprietary J-Brain Cargo® blood-brain barrier (BBB) penetration technology for lysosomal storage disorders (LSDs).

Under the terms of the agreement, the two companies will collaborate on research activities with an initial focus on LSD disease targets. Takeda also has the option to nominate additional rare disease and other disease indications at any time during the research period. Takeda will be responsible for further development and commercialization of any gene therapies developed under this collaboration. JCR will receive an immediate upfront payment from Takeda and has the right to receive further research, development and commercial milestones and royalties on post-approval sales. Takeda will also pay a nomination fee for each additional indication nominated under the agreement beyond the initially focused targets.

The agreement opens a new avenue for JCR to explore the potential applicability of J-Brain Cargo® to other modalities such as gene therapy. Furthermore, evaluating the usefulness of J-Brain Cargo® in a broad spectrum of disease conditions beyond LSDs may potentially lead to the discovery of new treatment options.

“JCR is accelerating efforts to create new value through multiple modalities,” said Shin Ashida, President and Chairman of JCR. “Through this agreement with Takeda, we seek to expand the possibilities of gene therapy using J-Brain Cargo® to develop new gene therapy treatments. I’m pleased to have this opportunity to further our collaboration with Takeda, a highly reputable partner in gene therapy research, and to expand our commitment to making a positive contribution to patients living with lysosomal storage disorders.”

“We are committed to advancing novel approaches through research partnerships and our internal expertise that scientifically demonstrate the greatest potential to improve treatment options for patients living with rare diseases,” said Madhu Natarajan, Head of the Rare Diseases Drug Discovery Unit at Takeda. “By combining JCR’s BBB crossing technology with the foundation Takeda is building in gene therapy drug development, we have the opportunity to create a portfolio of gene therapies designed to achieve broad biodistribution including in the central nervous system with the potential to halt or significantly slow progression of the somatic and neuropathic manifestations of lysosomal storage disorders.”

The impact of this exclusive license and collaboration agreement on the consolidated business results for the fiscal year ending March 31, 2022 has been incorporated into the business forecast for 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 46-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, MPS II (Hunter syndrome), 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), Hunter syndrome, Pompe disease, 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. 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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