



March 28, 2023
JCR Pharmaceuticals Co., Ltd.

Translation

JCR Pharmaceuticals Announces Achievement of Preclinical Proof-of-Concept Milestone Using J-Brain Cargo® Technology for Lysosomal Storage Disorder in Gene Therapy Collaboration with Takeda

Hyogo, Japan, Mar. 28, 2023 – [JCR Pharmaceuticals Co., Ltd.](https://www.jcrpharm.co.jp/en/site/en/) (TSE 4552; Chairman and President: Shin Ashida; “JCR”) today announced it has achieved the preclinical proof-of-concept milestone criterion in a collaboration with Takeda to develop gene therapies that apply JCR’s proprietary blood-brain barrier penetration technology, J-Brain Cargo®, to the treatment of lysosomal storage disorders (Related release is [here](#)).

The achievement of a predefined research milestone triggers a milestone payment to JCR. Under the terms of the agreement, JCR is eligible to further research, development, and commercial milestones and royalty payments on post-approval sales. The impact of this milestone payment on the consolidated business results for the fiscal year ending March 2023 has been incorporated into the business forecast for the current fiscal year.

“We are delighted with the progress in our collaboration with Takeda,” said Hiroyuki Sonoda, Ph.D., Vice President and Executive Director, Research Division. “This achievement demonstrates the applicability of the J-Brain Cargo® technology for the development of gene therapies for the treatment of lysosomal storage disorders and represents an important cornerstone to expand the application of J-Brain Cargo® technology to other modalities. We look forward to making advances in this collaboration with Takeda in line with JCR’s commitment to bringing various treatment options to individuals affected with lysosomal storage disorders.”

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 48-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. 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/>.

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