



December 20, 2023
JCR Pharmaceuticals Co., Ltd.

Translation

JCR Pharmaceuticals and Takeda Pharmaceuticals Announce Discontinuation of Gene Therapy Collaboration

Hyogo, Japan, Dec. 20, 2023 – [JCR Pharmaceuticals Co., Ltd.](#) (TSE 4552; Chairman and President: Shin Ashida; “JCR”) announced today that Takeda Pharmaceutical Co., Ltd. (“Takeda”) decided to discontinue their collaboration with JCR to develop gene therapies using adeno-associated viruses (AAV) combined with the JCR J-Brain Cargo® Technology.

Under the agreement signed on March 28, 2022, JCR and Takeda collaborated on the research and development of AAV-mediated gene therapies that apply JCR’s J-Brain Cargo® technology. In March 2023, JCR received a preclinical proof-of-concept milestone payment for confirming that the technology is able to accomplish therapeutically relevant effects in the brain of a murine disease model.

The discontinuation is due to the change in Takeda’s previously announced Research & Development (R&D) strategy to discontinue R&D on AAV-mediated gene therapy approaches. With the termination of the agreement, the results and patents have been returned to JCR. Following termination of the agreement, JCR plans to continue its own research efforts as well as engage with other companies on the application of the J-Brain Cargo® platform technology to gene therapies.

“Through our collaboration, we secured proof-of-concept for a gene therapy using our proprietary J-Brain Cargo® technology platform,” said Shin Ashida, Chairman and President of JCR. “We are grateful to Takeda for the partnership, and we understand and respect Takeda’s decision to change its R&D focus away from AAV-mediated gene therapies. We will continue to advance our own research and development in the field of gene therapy with a focus on collaborating with other companies thereby expanding outside of lysosomal storage disorders into additional neurological diseases.”

This termination is expected to have a minor impact on our consolidated financial results for this fiscal year ending on March 31, 2024.

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. Our approved products in Japan include therapies for the treatment of growth disorder, 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 (Hunter syndrome), MPS III A and B (Sanfilippo syndrome types 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. 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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