



October 31, 2022
JCR Pharmaceuticals Co., Ltd.

Translation

JCR to Initiate Development of New Drug Candidate for Fucosidosis

Oct. 31, 2022 -- [JCR Pharmaceuticals Co., Ltd.](#) (TSE 4552; Chairman and President: Shin Ashida; “JCR”) announced today its decision to develop a new drug candidate, JR-471, a blood-brain barrier (BBB)-penetrating α -L-fucosidase for the treatment of patients with fucosidosis, by applying J-Brain Cargo[®], JCR’s proprietary BBB penetrating technology.

This new target indication, fucosidosis is a lysosomal storage disorder that is inherited in an autosomal recessive manner. Mutations result in malfunction of a glycoprotein-metabolizing enzyme (α -fucosidase) which causes glycans and glycoproteins to accumulate throughout the body. Patients with fucosidosis display a variety of symptoms, including psychomotor symptoms, muscle hypotonia, visceromegaly, and skeletal abnormalities.

The symptoms of fucosidosis can vary greatly. The disease can be classified in the rapidly progressive form, causing severe, life-threatening complications in children or in the mild form develop during adolescence and with slower progression, but causing serious complications in adulthood. Previously, fucosidosis was classified as type I or II. However, fucosidosis is now more considered as a disease spectrum with a wide variety of expression. Fucosidosis type I (early onset, rapid progression) represents the severe end of the spectrum and type II the milder form. The prevalence of fucosidosis is very low, affecting less than 1 in 2 million people. There is no therapy available worldwide, and it is one of the ultra-rare diseases for which the development of a therapy is urgently awaited.

JCR has already obtained preliminary results using disease-model mice in which intravenously administered JR-471, demonstrated brain delivery and reduction of the disease-causing substrates in the brain. These results are consistent with the data from a series of drug candidates for lysosomal storage disorders including IZCARGO[®], which is the first drug that received regulatory approval in Japan for the treatment of MPS II, based on the J-Brain Cargo[®] technology for the delivery of therapeutics into the CNS. Based on the preclinical data, it is expected that JR-471 will have an adequate development success rate.

JCR has established an industry-leading development pipeline of therapeutic enzymes for LSDs that harness J-Brain Cargo[®] technology. As a specialty pharma devoted to the development of pharmaceutical products for rare diseases, JCR will accelerate its research and development activities to contribute to the treatment more patient populations suffering from LSDs.

This new drug development is expected to have a minor impact on JCR’s consolidated financial results for this fiscal year ending on March 31, 2023.

Lysosomal storage disorders (LSDs)

LSDs are designated as intractable diseases by the Ministry of Health, Labour and Welfare. They are also classified as chronic pediatric diseases of specific categories. LSDs are diseases in which genetic defects or mutations in hydrolytic enzymes, membrane proteins that serve as oxygen transporters and other proteins within lysosomes, which are organelles found in cells, result in the accumulation of substrates that cannot be decomposed within the lysosomes. This condition causes disorders in cells and tissues. There is a wide range of clinical symptoms that can appear depending on the specific substrates that accumulate. CNS disorders are present in many cases of LSDs.

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), 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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