



March 18, 2022  
JCR Pharmaceuticals Co., Ltd.

Translation

## **JCR to Initiate Development of New Drug Candidate for GM2 gangliosidosis**

Mar. 18, 2022 -- [JCR Pharmaceuticals Co., Ltd.](#) (TSE 4552; Chairman and President: Shin Ashida; “JCR”) announced today its decision to develop a new drug candidate, JR-479, a blood-brain barrier (BBB)-penetrating  $\beta$ -Hexosaminidase A for the treatment of patients with GM2 gangliosidosis, to which J-Brain Cargo<sup>®</sup>, JCR’s proprietary BBB penetrating technology, is applied.

This new target indication, GM2 gangliosidosis, is a lysosomal storage disorder (LSD) caused by deficiency in an enzyme,  $\beta$ -Hexosaminidase A. This results in accumulation of, GM2 ganglioside glycolipids in the central nervous system (CNS) and other organs of the body, and primarily causing progressive CNS symptoms. GM2 gangliosidosis is classified into infantile, juvenile and adult forms; the infantile form is the most severe, frequently leading to death within the first decade of life. There is no clinically established treatment worldwide for this disease, hence development of a new therapeutic agent that can reach the brain tissues is urgently anticipated.

$\beta$ -Hexosaminidase A, an enzyme that catabolizes GM2 ganglioside, is a molecule composed of  $\alpha$ - and  $\beta$ -subunits. Deficiency in the  $\alpha$ -subunit is classified as Tay-Sachs, while  $\beta$ -subunit deficiency is classified as Sandhoff disease. JR-479 has been designed to address both Tay-Sachs and Sandhoff disease. The application of J-Brain Cargo<sup>®</sup> is expected to enable efficient enzyme delivery to the brain tissues to reduce abundant causative substrate accumulations.

Animal studies with intravenously administered JR-479 demonstrated its brain delivery and subsequent reduction in the causative substrates. JCR is moving forward to devise developmental plans aiming to initiate clinical trials within three years.

JCR has conducted a series of development of therapeutic enzymes for LSDs that harness J-Brain Cargo<sup>®</sup> 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 treatment for a broader patient population.

This new drug development is expected to have a minor impact on our consolidated financial results for this fiscal year ending on March 31, 2022.

### **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 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, MPS IIIA and B (Sanfilippo disease), 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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