



September 14, 2018
JCR Pharmaceuticals Co., Ltd.

Translation

JCR to Initiate Development of a New Drug Candidate for Sanfilippo Syndrome Type A Using J-Brain Cargo®

JCR Pharmaceuticals Co., Ltd. (TSE 4552; Chairman and President: Shin Ashida; “JCR”) announced today of its decision to develop a new drug candidate, JR-441, a blood-brain barrier (BBB)-penetrating therapeutic enzyme (heparan N-sulfatase) for the treatment of patients with Sanfilippo syndrome type A, to which J-Brain Cargo®, JCR’s proprietary BBB penetration technology, is applied.

Sanfilippo syndrome type A (mucopolysaccharidosis type IIIA) belongs to mucopolysaccharidosis, of which Hunter Syndrome and Hurler syndrome are well-known subtypes. It is characterized by the deficiency of a specific enzyme called heparan N-sulfatase, which is responsible for the degradation of heparan sulfate. This deficiency results in accumulation of heparan sulfate, leading to various symptoms including central nervous system (CNS) disorders, sleep disorders, hepatosplenomegaly and seizures. Presently, no effective treatment for Sanfilippo syndrome type A is available, thus development of a new treatment option has been long awaited for this intractable disease.

The animal studies with JR-441 demonstrated delivery of JR-441 not only into peripheral tissues but also into the brain, along with highly effective reductions in the heparan sulfate accumulations in these tissues. Given such promising results, JCR will compile a development plan for JR-441 with an aim toward an early initiation of clinical trials.

JCR has focused on developing a robust pipeline of innovative therapeutic enzymes for lysosomal storage disorders harnessing J-Brain Cargo® technology. We will develop JR-441 as a new drug candidate with an application of the BBB penetration technology, following JR-141 for Hunter syndrome, JR-162 for Pompe disease and JR-171 for Hurler syndrome. As a specialty pharma devoted to the development of pharmaceutical products for rare diseases, JCR will strive to contribute to treatment available for broader patient population.

This new drug development is expected to have a minor impact on JCR’s consolidated financial results for the year ending March 31, 2019, whilst it is expected to add to our future revenues.

Lysosomal storage disorders (LSDs)

LSDs are designated as an intractable disease 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 broken down within the lysosomes. This condition causes disorders in cells and tissues. There are a wide range of clinical symptoms that can appear depending on the specific substrates that accumulate. CNS disorders are present in almost all of cases of LSDs.

Sanfilippo syndrome type A (mucopolysaccharidosis type IIIA)

Sanfilippo syndrome type A is designated as an intractable disease by the Ministry of Health, Labour and Welfare. It is classified as a form of mucopolysaccharidosis, which is one of the lysosomal storage disorders. Sanfilippo syndrome type A is caused by an inborn deficiency or defect in heparan N-sulfatase within lysosomes in cells throughout the body. The main symptoms are CNS disorders, sleep disorders, hepatosplenomegaly and seizures. Sanfilippo syndrome is divided into four different types (type A to type D). At present, effective treatment of Sanfilippo syndrome is not available.

[About JCR Pharmaceuticals]

JCR is a specialty pharma engaged in the research, development, manufacture and marketing of biopharmaceuticals and regenerative medicine with a focus on rare diseases. Its philosophy, "Contributing towards people's healthcare through pharmaceutical products" drives JCR to create innovative pharmaceutical products as value-added treatment options for the under-served patient community.

[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 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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