September 21, 2018
JCR Pharmaceuticals Co., Ltd.

Translation

JCR Receives Marketing Approval for Agalsidase Beta BS I.V. Infusion [JCR] for Fabry Disease

JCR Pharmaceuticals Co., Ltd (TSE: 4552, Chairman and President: Shin Ashida, "JCR") announced today that it has received marketing approval from the Ministry of Health, Labour and Welfare of Japan for its Agalsidase Beta BS I.V. Infusion 5mg and 35mg [JCR] (also known as JR-051).

Agalsidase Beta BS I.V. Infusion [JCR] is developed as a biosimilar for agalsidase beta for the treatment of Fabry disease, a type of lysosomal storage disorders (LSDs). This product is not only JCR's first enzyme replacement therapy (ERT) for LSDs, but also the first domestic ERT product for LSDs. Clinical trials have shown Agalsidase Beta BS I.V. Infusion [JCR] having the same efficacy and safety profiles as the innovator product agalsidase beta (genetic recombination). As JCR has realized high-quality manufacturing through its serum-free culture technology, Agalsidase Beta BS I.V. Infusion [JCR] is expected to become a new treatment option for Fabry disease.

JCR has focused on developing a robust pipeline of innovative therapeutic enzymes for LSDs harnessing J-Brain Cargo®, JCR's proprietary BBB penetration technology. 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.

In terms of impact on JCR's consolidated financial results for the fiscal year ending March 31, 2019. The figures in the forecast will remain unchanged as this item was factored into planning at the beginning of the fiscal year.

Agalsidase Beta BS I.V. Infusion [JCR]

- Product Name: Agalsidase Beta BS I.V. Infusion 5mg
  Agalsidase Beta BS I.V. Infusion 35mg
- Generic Name: Agalsidase beta (rDNA origin)
- Indications: Fabry disease (a Ministry of Health, Labour and Welfare-designated intractable disease)
- Dosage and administration: An intravenous infusion administered once every other week at a dose of 1.0 mg per 1 kg of body weight

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.

Fabry disease
Designated as intractable by Japan's Ministry of Health, Labour, and Welfare, Fabry disease is a type of LSDs and a congenital sphingolipidosis that results from a deficiency or defect of the lysosomal hydrolytic enzyme alpha-galactosidase A (α-GAL) in the body's cells. Main symptoms include pain in the extremities, corneal opacity, renal and cardiac dysfunctions. Currently available standard therapy is ERT with a therapeutic enzyme administered intravenously every two weeks.

[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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