



28 November, 2018
JCR Pharmaceuticals Co., Ltd.

Translation

Launch of Agalsidase Beta BS I.V. Infusion [JCR] for Fabry Disease

JCR Pharmaceuticals Co., Ltd (TSE: 4552, Chairman and President: Shin Ashida, “JCR”) announces today that Agalsidase Beta BS I.V. Infusion 5mg and 35mg [JCR], approved in September 2018, is being placed on the National Health Insurance (NHI) reimbursement price list and launched as of today.

This product is not only JCR’s first enzyme replacement therapy (ERT) product for lysosomal storage disorders (LSDs), but also the first domestic therapeutic enzyme for LSDs based on the serum-free culture technology. Agalsidase Beta BS I.V. Infusion [JCR] is expected to become a new treatment option for Fabry disease.

JCR has been focusing on the development of a robust pipeline of innovative therapeutic enzymes for LSDs. As a specialty pharma devoted to the pharmaceutical products for rare diseases, JCR will strive to contribute to treatment available for a 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.



(left) Agalsidase Beta BS I.V. Infusion 5mg

(right) Agalsidase Beta BS I.V. Infusion 35mg

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
- Approval date: September 21, 2018
- NHI reimbursement price listing date: November 28, 2018
- NHI reimbursement price: 83,921 yen per bottle of 5mg, 469,292 yen per bottle of 35mg

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 enzyme 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 as the enzyme activity decreases. 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 most 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 cells throughout the whole body. 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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