News Release

June 17, 2020
JCR Pharmaceuticals Co., Ltd.

Translation

Agalsidase Beta BS I.V. Infusion [JCR] (JR-051) for Fabry Disease:
Notice on the Publication of the Results of the Phase 1 and 2/3 Clinical Trials in *Molecular Genetics and Metabolism*

JCR Pharmaceuticals Co., Ltd. (TSE 4552; Chairman and President: Shin Ashida; "JCR") announced today that the results of the phase 1 and 2/3 clinical trials of Agalsidase Beta BS I.V. Infusion [JCR] (JR-051), recombinant Agalsidase Beta, for Fabry disease have been published in the electronic edition of *Molecular Genetics and Metabolism*, the official journal of *Society for Inherited Metabolic Disorders*. This is JCR's first product for enzyme replacement therapy (ERT) for Lysosomal Storage Disorders (LSDs), also the first of the kind manufactured in Japan. Agalsidase Beta BS I.V. Infusion [JCR] has been launched since November 2018 as the first biosimilar for the treatment of rare diseases. A summary of the article is as follows.

◆ Title:
Pharmacokinetics and pharmacodynamics of JR-051, a biosimilar of agalsidase beta, in healthy adults and patients with Fabry disease: Phase I and II/III clinical studies

◆ Digital Object Identifier: https://doi.org/10.1016/j.ymgme.2020.04.003

◆ Summary
The Phase 1 and 2/3 studies were conducted with the aim to verify clinical comparability of JR-051 and an upfront biopharmaceutical (agalsidase beta). The results demonstrated that JR-051 and agalsidase beta are comparable in terms of efficacy and safety.

【Phase 1 study】
20 healthy adult male volunteers were administered JR-051 and agalsidase beta to confirm pharmacokinetic equivalence in a randomized, double-blind, parallel-group manner. The study demonstrated comparable pharmacokinetic profiles of JR-051 and agalsidase beta.

【Phase 2/3 study】
16 patients with Fabry disease underwent treatment with agalsidase beta (1mg/kg, once every other week), then were switched to intravenous administrations of JR-051 (1 mg/kg, once every other week).

・Efficacy : The 95% confidence intervals of the ratios of the GL-3 plasma concentrations (primary endpoint) during the agalsidase beta treatment, as well as those of Lyso-GL-3, to the respective plasma concentrations after 26 and 52 week-administrations of JR-051 were within pre-determined equivalence acceptability ranges.

・Safety：No severe infusion associated reactions (IARs), such as anaphylactic shock, were observed. One IAR, commonly observed with the ERT for Fabry disease, was reported in a patient after JR-051 administration.

[About JCR Pharmaceuticals]

JCR is a specialty pharma company engaged in the research, development, manufacturing 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 populations.

[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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