



February 13, 2018
JCR Pharmaceuticals Co., Ltd.

Translation

JCR Announces Data Presentation on Non-clinical and Phase II/III Study Results of JR-051 for Fabry Disease at the 14th Annual *WORLDSymposium*TM 2018

JCR Pharmaceuticals Co., Ltd. (TSE 4552: Chairman and President Shin Ashida, "JCR") announced two poster presentations on non-clinical and phase II/III study results of JR-051 (recombinant alpha-galactosidase A), a therapeutic enzyme for Fabry disease under application for marketing approval as a biosimilar to recombinant agalsidase beta. The summary of the presentations at the 14th Annual *WORLDSymposium*TM 2018* in San Diego (February 5-9, 2018) is shown below.

Fabry disease is a lysosomal storage disorder caused by an inherited alpha-galactosidase A (α -GalA) gene mutation resulting in an absence or decrease in α -GalA activity and accumulation of undegraded substrate, GL-3, in lysosomes. Currently under development, JR-051 is a domestic drug product produced in serum-free cultivation.

[Non-clinical studies]

Efficacy and tissue distribution comparative studies using α -GalA gene knockout mice, a Fabry disease mouse model, and toxicological studies using monkeys demonstrated that the pharmacological and pharmacokinetic properties and safety of JR-051 are equivalent to an innovator product (agalsidase beta [recombinant]).

[Phase II/III study]

The equivalent efficacy of JR-051 to the innovator product was verified in phase II/III study with Fabry patients. Also, the safety of JR-051 was confirmed.

- The study demonstrated the equivalence in efficacy of JR-051 and the innovator product based on plasma GL-3 concentrations before and 26 weeks after administration of JR-051 as the indicator. In addition, it was confirmed that the efficacy is equivalent to the innovator product even with a plasma lyso-GL-3 concentration as the indicator.
- The similarity in the safety profile of JR-051 to the innovator product was confirmed.

Based on the results in above-mentioned non-clinical and phase II/III studies, JR-051 is expected to be a new treatment option for Fabry disease.

**WORLDSymposium*TM is an international symposium held annually in the United States with a focus on the basic research to clinical application in the lysosomal diseases.

<http://www.worldsymposia.org/>

[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.

Investors & Media:

JCR Pharmaceuticals Co., Ltd.

Corporate Communications

ir-info@jcrpharm.co.jp

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