



January 27, 2021
JCR Pharmaceuticals Co., Ltd.

Translation

JR-141 (Pabinafusp Alfa) for Hunter Syndrome: Notice on the Publication of the Results of non-clinical Trials in Molecular Therapy

January 27, 2021 -- JCR Pharmaceuticals Co., Ltd. (TSE 4552; Chairman and President: Shin Ashida; "JCR") announced today that the results of the non-clinical trials of JR-141 (Pabinafusp Alfa) with MPS II mice for the treatment of mucopolysaccharidosis II (MPS II; Hunter syndrome) have been published in the electronic edition of [Molecular Therapy](#), the official journal of [American Society of Gene and Cell Therapy](#).

This content will be presented at the 17th Annual *WORLDsymposium™* 2021 (February 8-12, 2021(EST), virtual-only).

A summary of the article is as follows.

◆ Title:

Clearance of heparan sulfate in the brain prevents neurodegeneration and neurobehavioral abnormality in mucopolysaccharidosis II mice

◆ Digital Object Identifier: <https://doi.org/10.1016/j.ymthe.2021.01.027>

◆ Summary

The fusion protein pabinafusp alfa (investigational code name, JR-141) was intravenously administered to the MPS II mice once weekly at 2 mg/kg or once biweekly at 4 mg/kg for 36 weeks. Pabinafusp alfa with either regimen markedly reduced heparin sulfate (HS) and attenuated histopathological changes in the brain. Loss of spatial learning ability was completely suppressed by the chronic treatment with pabinafusp alfa but not with the comparator. HS concentration in the cerebrospinal fluid (CSF) highly correlated with that in the brain.

In conclusion, repeated intravenous administration of pabinafusp alfa decreased HS deposition in the brain, leading to the prevention of neurodegeneration and the maintenance of neurocognitive function, which may be predictable from reduction of HS concentrations in the CSF.

[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 are 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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