



July 28, 2022  
JCR Pharmaceuticals Co., Ltd.

Translation

## **JCR files for expanded use GROWJECT® for short stature due to SHOX-deficiency**

July 28 -- JCR Pharmaceuticals Co., Ltd. (TSE 4552; Chairman and President: Shin Ashida; “JCR”) announced today that JCR filed a partial change application for supplemental indication in Japan with GROWJECT® (somatropin), a recombinant human growth hormone product, for the treatment of short stature due to Short Stature Homeobox-containing Gene (SHOX) deficiency.

SHOX deficiency is a congenital disorder caused by micro deletions, duplications, or mutations that result in loss of function of a growth-gene *SHOX* (Short Stature Homeobox-containing Gene) present on the sex chromosomes.

This disease presents with growth failure due to an underlying genetic alteration, and there are no approved drugs available in Japan for the treatment of SHOX-deficiency.

This application is based on the results of a phase III clinical trial conducted in Japan (related release is [here](#)). This trial found a statistically significant difference between the GROWJECT® group and the untreated group on the primary endpoint, change in height SDS (over 12 months from baseline). There were no serious adverse events related to the drug.

GROWJECT®, one of JCR’s core products, is approved and launched for the treatment of short stature due to pediatric growth hormone deficiency, turner syndrome and short body height for gestational age, and for adult growth hormone deficiency in Japan.

Currently, JCR is also developing a long-acting growth hormone product for short stature due to pediatric growth hormone deficiency (related release is [here](#)). JCR will continue to strive for provision of various treatment options to meet the needs of the patients suffering from growth disorders.

This application is expected to have a minor impact on consolidated financial results for the year ending on March 31, 2023.

### **About JCR Pharmaceuticals Co., Ltd.**

JCR Pharmaceuticals Co., Ltd. (TSE 4552) is a global specialty pharmaceuticals company that is redefining expectations and expanding possibilities for people with rare and genetic diseases worldwide. We continue to build upon our 47-year legacy in Japan while expanding our global footprint into the US, Europe, and Latin America. We improve patients’ lives by applying our

scientific expertise and unique technologies to research, develop, and deliver next-generation therapies. Our approved products in Japan include therapies for the treatment of growth disorder, Fabry disease, acute graft-versus host disease, and renal anemia. Our investigational products in development worldwide are aimed at treating rare diseases including MPS I (Hurler, Hurler-Scheie and Scheie syndrome), MPS II (Hunter syndrome), MPS IIIA (Sanfilippo A), MPS IIIB (Sanfilippo B), and more. JCR strives to expand the possibilities for patients while accelerating medical advancement at a global level. Our core values – reliability, confidence, and persistence – benefit all our stakeholders, including employees, partners, and patients. Together we soar. For more information, please visit <https://www.jcrpharm.co.jp/en/site/en/>.

### **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.*

#### Contact:

Investors & Media:

JCR Pharmaceuticals Co., Ltd.

Corporate Communications

[ir-info@jcrpharm.co.jp](mailto:ir-info@jcrpharm.co.jp)

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