**News Release** 



June 24, 2015 JCR Pharmaceuticals Co., Ltd.

Translation

# Announcement of Mid-Term Management Plan (FY2015-FY2019) – "HIYAKU" –

JCR Pharmaceuticals Co., Ltd. (Headquarters: Ashiya, Hyogo; Chairman and President Shin Ashida) ("JCR") announced today the five-year mid-term management plan for fiscal years ("FY") 2015 through FY2019, ending March 31, 2020 ("the Mid-Term Plan").

In this Mid-Term Plan, we have chosen the word "HIYAKU" (leap into the future) for its key concept as technologies and experiences accumulated to date are beginning to yield results and we are ready to advance toward the next stage. Furthermore, we have selected the following four focal points: (i) Advancing R&D activities one step beyond, (ii) reinforcing our capabilities via new businesses development, (iii) further enhancing "Business Structure" and "Product Strategy", and (iv) reinforcing "Management Platform" in order to become an R&D oriented specialty pharma with global exposure.

# Overview of the Mid-Term Plan

1. Vision of JCR

We aim to become an R&D oriented specialty pharma with global exposure built on proprietary biotechnologies and technologies supporting cell therapy and regenerative medicine which we have been developing since foundation.

2. Key concept

"HIYAKU" – Now is the time to leap into the future.

3. Focal points to achieve our goals

In order to advance to become an R&D oriented specialty pharma with global exposure, we will tackle the following focal points:

- (i) Advancing R&D activities one step beyond
- (ii) Reinforcing our capabilities via new businesses development
- (iii) Further enhancing "Business Structure" and "Product Strategy"
- (iv) Reinforcing "Management Platform"

4. Numerical targets on a consolidated basis

In FY2019, which is the last fiscal year covered by the Mid-Term Plan, we aim to achieve sales of 25 billion yen and increase operating profit to 5 billion yen by absorbing the increase in R&D expenses due to the progress of R&D projects.

|                     | FY2014 (Actual results) | FY2019 (Goal)  |
|---------------------|-------------------------|----------------|
| Sales               | 16.8 billion yen        | 25 billion yen |
| Operating<br>Profit | 2 billion yen           | 5 billion yen  |

- 5. Future business development
  - We will utilize our proprietary technologies for developing recombinant proteins, namely "J-MIG System", "J-GlycoM" and "J-GlycoS" and launch new biopharmaceuticals in Growject® and ESA businesses and for rare diseases.
  - We will strive for continuous earnings expansion by launching JR-031 (mesenchymal stem cells), the first domestic regenerative medical product of allogeneic cells, for which marketing approval is anticipated by the end of this year.
  - For new business development, we will promote our licensing business which includes licensing-out of our proprietary technologies, such as "J-Brain Cargo", a BBB penetration technology, to Japanese and overseas companies.
  - We will encourage global marketing of in-house developed products manufactured under our global standard production and quality assurance system through alliances with partners.
  - We will make cell therapy and regenerative medicine technologies a new R&D pillar and work on the development and creation of regenerative medical product following JR-031.

For details, please see the reference material to be released on June 29.

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# [Glossary]

# J-MIG System

This refers to a high-level CHO cell expression system, which is one of our proprietary technologies. This "Modified-IRES-GS Lineage High-level Expression System" (J-MIG System) preferentially amplifies the incorporated target DNA in order to express target protein efficiently in CHO cells. The presentation our researchers made in relation to J-MIG System at "IBC's 10th Annual Cell Line Development & Engineering" held on September 8, 2014 in the U.S. won the first prize.

# J-GlycoM, J-GlycoS

These are technologies to obtain glycoproteins with desired sugar chain structures when producing recombinant proteins that were established in the course of our development of biopharmaceuticals over the years. Specifically, J-GlycoM is a technology for expressing glycoproteins having only mannose-type sugar chains and J-GlycoS for expressing glycoproteins highly-modified with sialic acids in an ingenious cultivation process.

#### <u>ESAs</u>

ESAs refer to erythropoiesis-stimulating agents. ESAs include "Epoetin Alpha BS Inj.", a therapeutic product for renal anemia manufactured by JCR and marketed by JCR and Kissei Pharmaceutical Co., Ltd., and JR-131, a long-acting therapeutic product for renal anemia currently under development.

#### Regenerative medical products

Regenerative medical products are a newly defined category that was established in addition to existing pharmaceutical products, medical devices, etc. in the new PMD Act ("Act on Securing Quality, Efficacy, and Safety of Pharmaceuticals, Medical Devices, Regenerative and Cellular Therapy Products, Gene Therapy Products, and Cosmetics") enforced on November 25, 2014 following the revision of the Pharmaceutical Affairs Law. Regenerative medical products include gene therapy products, processed cells used in cancer immunotherapies and oncolytic viruses in addition to human cells, tissues, etc. processed or prepared for transplantation that are assumed to be used in traditional regenerative medicine.

#### **BBB (Blood-Brain Barrier)**

The BBB is a barrier function restricting the penetration of substances in blood into the brain and

indispensable for the maintenance of its homeostasis. This function is performed by vascular endothelial cells that cover the inside of brain capillaries lining so close to each other and forming a tight junction. It is difficult for high molecules such as proteins to penetrate this barrier and, as a result, the BBB can impede the successful delivery of pharmaceutical products, including those for the treatment of Hunter syndrome, into the brain. Development of a penetration technology is much-awaited.

# J-Brain Cargo

High molecules such as proteins are usually unable to penetrate the BBB. J-Brain Cargo is our proprietary technology enabling the penetration.

#### [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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