



# Midterm Business Plan for 2020-2022 “REVOLUTION”

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JCR Pharmaceuticals Co., Ltd.

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**REVOLUTION**  
*into the Future*

## FORWARD-LOOKING STATEMENT

This presentation contains forward-looking statements that are subject to a number of risks and uncertainties, many of which are outside our control. All forward-looking statements regarding our plans, outlook, strategy and future performance are based on judgments derived from the information available to us at this time.

All forward-looking statements speak only as of the date of this presentation.

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.

This document is not intended to guarantee and advertise the efficacy of the product under development.

The progress of clinical development may be affected by the pandemic of novel coronavirus infection (COVID-19) in the future .

- **General Overview of “HIYAKU”  
(Previous Business Plan for FY2015-FY2019)**
- **Mid- to Long-Term Management Vision “Toward 2030”**
  - Corporate Philosophy
  - Mid- to Long-Term Management Vision
  - Core Values
  - History of Growth and Characteristics of Our R&D
  - Potential of Our Basic Technology
  - R&D Direction
  - R&D Timeline
- **Midterm Business Plan for FY2020-FY2022 “REVOLUTION”**
  - Keyword
  - Important Business Challenges
  - Guidance for 2022
  - JCR’s Sustainability

# **General Overview of “HIYAKU”**

## **(Previous Business Plan for FY2015-FY2019)**

# Achievements during Previous Business Plan

## FY2015

- Feasibility Study Agreement with Sumitomo Dainippon Pharma and Eisai for J-Brain Cargo®
- Application for Marketing Authorization of GROWJECT® Liquid Formulation
- Office nursery “JCR Kids Land”
- Approval and Launch of TEMCELL® HS inj.
- Research agreement with PeptiDream



## FY2016

- CTMC/CPC Completed
- Initiation of Darbepoetin Alfa BS Injection JCR PIII trial in Japan
- Approval and Launch of GROWJECT® Liquid Formulation
- Initiation of JR-141 PI/II trial in Japan



## FY2017

- Development Agreement with Teijin for JTR-161/JR-161 in Japan
- Business and Capital Alliance with MEDIPAL HOLDINGS
- Application for Marketing Authorization of Agalsidase Beta BS I.V. Infusion in Japan
- Research Agreement with Nanocarrier
- MoU with the Grand Duchy of Luxemburg on Leasing of industrial site
- License Agreement for J-Brain Cargo® with Sumitomo Dainippon Pharma
- Foundation of JCR USA
- Designation of JR-141 under “Sakigake”

## FY2018

- Initiation of Second Clinical Trial Material Manufacturing Center Construction
- Initiation of GROWJECT® PIII trial for SHOX deficiency in Japan
- Initiation of PIII trial in Japan and PII trial in Brazil for JR-141
- Orphan designation for JR-141 in US and EU
- Approval and Launch of Agalsidase Beta BS I.V. Infusion in Japan
- Application for Marketing Authorization of Darbepoetin Alfa BS Injection JCR in Japan
- Application for Marketing Authorization of TEMCELL® HS inj. for Epidermolysis Bellosa in Japan



## FY2019

- Initiation of JR-142 PI trial in Japan
- Creation of constrained peptide with PeptiDream
- Initiation of TEMCELL® HS inj. PI/II trial in Japan for neonatal hypoxic ischemic encephalopathy
- Expansion for Research Facilities
- Approval and Launch of Darbepoetin Alfa BS Injection JCR in Japan
- Agreement of an acquisition of Armagen, Inc.



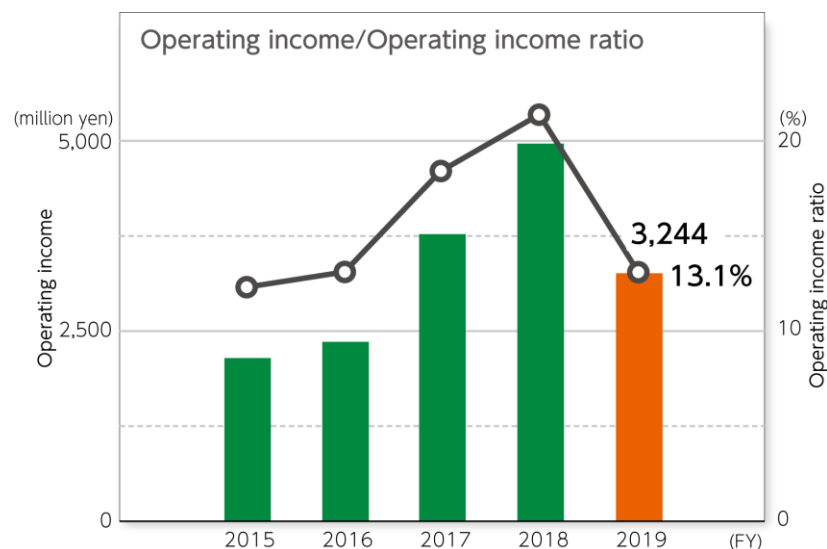
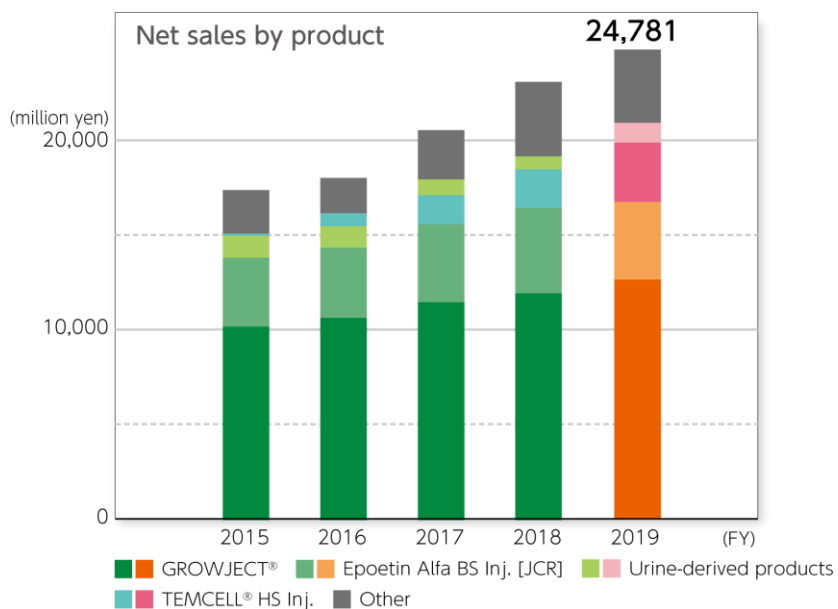
# Achievements during Previous Business Plan

## FY2015 to FY2019

Sales : ¥17.4 bil to ¥24.7 bil

Operating income : ¥2.15 bil to ¥3.24 bil

Operating income to sales : 12.3% to 13.1%



# Achievements during Previous Business Plan

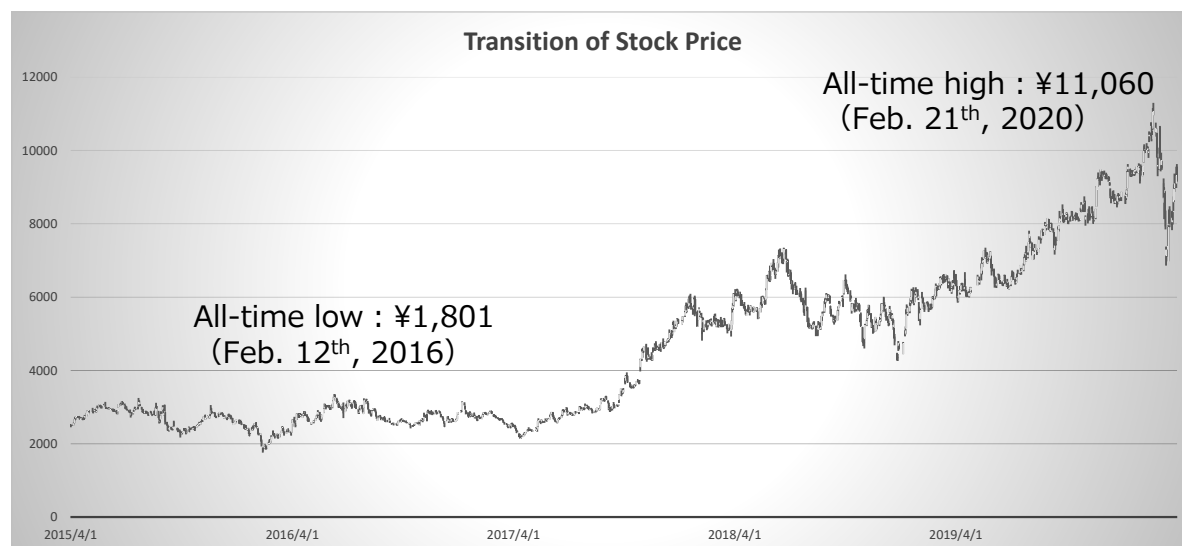
FY	Num. Employees (Non-consolidated)	Avg. Age	Duration of Service	Avg. Annual Salary
2019	654	41.4	8.9	¥8.3 mil
2018	613	41.4	8.8	¥8.3 mil
2017	540	41.6	9.0	¥8.2 mil
2016	522	41.4	8.6	¥8.1 mil
2015	487	41.1	8.3	¥8.1 mil

## As of Mar. 31, 2019

No. of Employees : 654  
 Avg. age : 41.4  
 Avg. salary : ¥8.3 mil

## As of Mar. 31, 2016

No. of Employees : 487  
 Avg. age : 41.1  
 Avg. salary : ¥8.1 mil



## As of Mar. 31, 2019

Close : ¥9,420  
 Market Cap. : ¥305.4 bil

## As of Apr. 1, 2015

Close : ¥2,480  
 Market Cap. : ¥80.4 bil

## » JCR achieved substantial results for the next stage during “HIYAKU” with “Speed First” spirit.

- Approval and launch of TEMCELL® HS inj. ,GROWJECT® liquid formulation, Agalsidase beta BS I.V. infusion and Darbepoetin alfa BS injection
- On-track progress of JR-141 PI/II trial in Japan and PII trial in Brazil
- PII trial in Brazil, the first global development for JCR, successfully initiated with the support of the patient advocacy group and medical experts
- Acquisition 100% ownership of ArmaGen Inc. to fortify the lysosomal enzyme replacement therapy (ERT) franchise
- Construction of CTMC (Clinical Trial Material Center) and its annex, CTMC2, for lysosomal drug candidates
- Construction of CPC (Cell Processing Center) for DPC (Dental Pulp Cell) regenerative medicine candidate

## » To strengthen licensing activities for rare disease business to maximize corporate value



Through our business activities centered on the field of pediatric rare diseases, we will strive to “contribute to unmet medical needs” and enhance activities related to “Rare Diseases: RD, ESG”.

## Initiatives



### Rare Diseases (RD)

Conduct awareness-raising activities as a company promoting research and development of orphan drugs.



Launched the "RARE DISEASE" project, with “What JCR can do” as its motto, promote awareness-raising activities and initiatives for Rare Disease Day.



### Environment (E)

Implemented efforts to reduce the environmental impact of business activities.



### Society (S)

Maintain a comfortable working environment and conduct initiatives aimed at society.



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To contribute to addressing the unmet medical needs of patients all over the world, we support the GLOBAL FOUNDATION FOR LIFE SCIENCE (a non-profit foundation in Switzerland), the Mother and Child Health Encouragement Award, and Momiji House (a short-term residential facility for severely ill children and their families).



### Corporate Governance (G)

Establish and operate a system to maintain legality, transparency and objectivity of management.

Contribution  
through our business

Promote the “Realizing medical care for those living with rare diseases”

# Mid-to Long-Term Management Vision “Toward 2030”



Research-Oriented  
Specialty Pharma  
with  
Global Exposure

Core Value  
“Team JCR”

**LEAP**  
into the Future

**REVOLUTION**  
into the Future

# Corporate Philosophy

**JCR is committed to challenging rare diseases by uniting our R&D and manufacturing capabilities to contribute to the patients and children around the world.**

**Contributing towards people's healthcare  
through pharmaceutical products**

## Reliability

We strive to establish a reliable company for all stakeholders by actions with high sense or duty in addition to compliance.

## Confidence

We take a unique approach in advancing our research and development and provide high-quality products and information with confidence in the aim of providing world-class pharmaceuticals.

## Belief

We aim for further corporate growth in the belief of "Think by oneself, act by oneself" under the basic philosophy.



## Our Goal

**Research-oriented specialty pharma with global exposure**

## Corporate Image in Detail

- ◎ **Global specialty pharma in the rare disease arena**
- ◎ **Continuing challenges to create “one step beyond” technologies based on our unique technology platform such as J-Brain Cargo®**
- ◎ **Continuing challenges to keep on creating new values with “R&D” and “Manufacturing”**
- ◎ **Continuing challenges with stalwart faith to contribute to rare diseases**

## Basic Strategy

- ◎ **Focus on “R&D” and “Manufacturing” with the foundation spirit of “Team JCR” as our source of values**
- ◎ **Cultivate human resources with “Team JCR” spirit so that they can flourish in each section**
- ◎ **Secure three pillars of business: Revenue from domestic products, Revenue from global market of ERT for lysosomal storage disease products and Revenue from licensing of our technologies**



## **“Team JCR” is our source of values.**

JCR’s growth has been driven by “R&D” and “Manufacturing” capabilities.

Each and every person who has been inspired by JCR’s philosophy has founded a robust business platform and created its various cutting-edge technologies including J-Brain Cargo® which is now the cornerstone of its forthcoming global business.

JCR, with sturdy certitude that its source of value is “Team JCR”, will promote its corporate culture in our global business era, will accelerate innovation by focusing on “R&D” and “Manufacturing”, and will contribute to rare diseases by creating new added value.

JCR, in principle, will not deploy its subsidiary for marketing products abroad, but will focus its resources on “R&D” and “Manufacturing” promoting its corporate culture, “Team JCR”.

JCR will value collaborations with other companies whose cultures are in tune with ours based on the certitude that mid-to long-term steady growth is coming from “Team JCR” spirit.

JCR, looking ahead to its globalization in the near future, will promote its “Team JCR” culture, will consolidate its robust management foundation which can make audacious and appropriate decisions in due time and will pursue steady growth in the growing uncertainty of the pharma business.

# History of Growth History and Characteristics of Our “R&D”

Purification technology cultivated since JCR's foundation, growth hormone business entered since 1985, and recombinant DNA and regenerative medical technologies engaged since 2000 contributed to the steady growth of JCR to date.

## From human-derived material

- Urokinase/Ulinastatin (urine)
- Epidermal Growth Factor (urine)
- Interferon (blood)
- Kallidinogenase (urine)
- Fibronectin (blood)
- Thrombomodulin (urine)

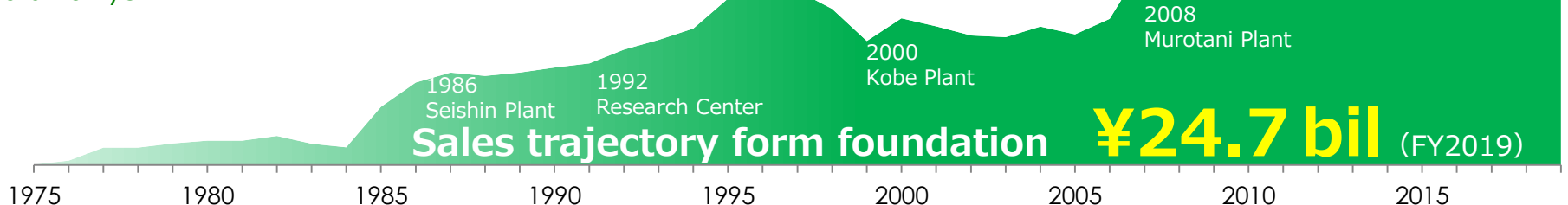
## From genetic recombination technology

- Epoetin Alfa BS inj. “JCR” (2010)
- Agalsidase Beta BS infusion. “JCR” (2018)
- Darbepoetin Alfa BS inj. “JCR” (2019)
- J-Brain Cargo® applied candidates including JR-141

## From regenerative technology

- TEMCELL® HS inj. (2016)
- JTR-161/JR-161
- Gene therapy technology

10 billion yen

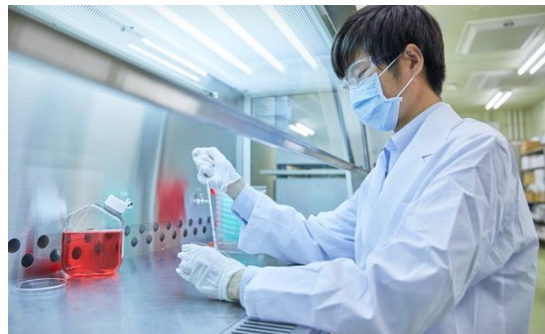


Purification tech.

Cell construction fermentation tech.

Foray to regenerative area

- Steady R&D activity with robust business base from growth hormone revenue (since 1993).
- Purification technology as our fundamental technology starting from urokinase business, cultivated through over 20 years of R&D using human-derived materials such as urine and blood.
- Recombination DNA technology since the early 2000s when JCR shifted to the area of its R&D focus. Our first product was the first domestic biosimilar in Japan, Epoetin Alfa BS launched in 2010.
- JCR has been engaging in the development of drugs for lysosomal storage diseases from early 2000s.
- Mesenchymal stem cell research since 2003. Our first product was the first allogenic cell product in the world, TEMCELL® HS, launched in 2016.  
JCR has been engaging in the development of dental pulp cells for acute cerebral infarction.
- Cutting-edge technologies J-Brain Cargo®, Modified albumin-based long-acting technology, J-Mig (High Expression system), and J-Glyco S/M (Glycosylation technology) resulting from thorough and repeated hypothesis verification.



**Effective drugs are desired for lysosomal storage diseases, a group of ultra-rare diseases.**

**With the development of our drug candidates utilizing JCR's proprietary Blood Brain Barrier (BBB)-penetrating technology, JCR expects potential expansion of market penetration.**

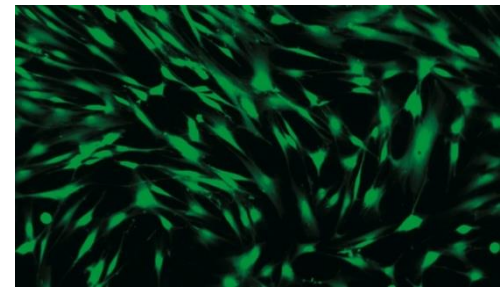
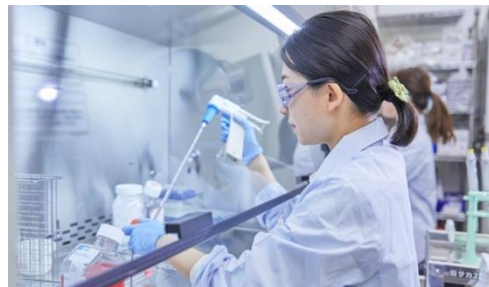
**Estimated number of patients and size of market for five lysosomal storage diseases**

Indication	Number of patients <sup>※1</sup>		Market size <sup>※2</sup>	
	Japan	World	Japan (2019)	World (2019)
MPS II (Hunter)	250	7,800	¥7.6 bil	¥87.0 bil
Pompe	80	10,600	¥3.0 bil	¥110.0 bil
MPS I (Hurler/Hurler Scheie/Scheie)	60	3,600	¥1.6 bil	¥28.0 bil
MPS IIIA (Sanfilippo A)	60	6,890	—	—
MPS IIIB (Sanfilippo B)				

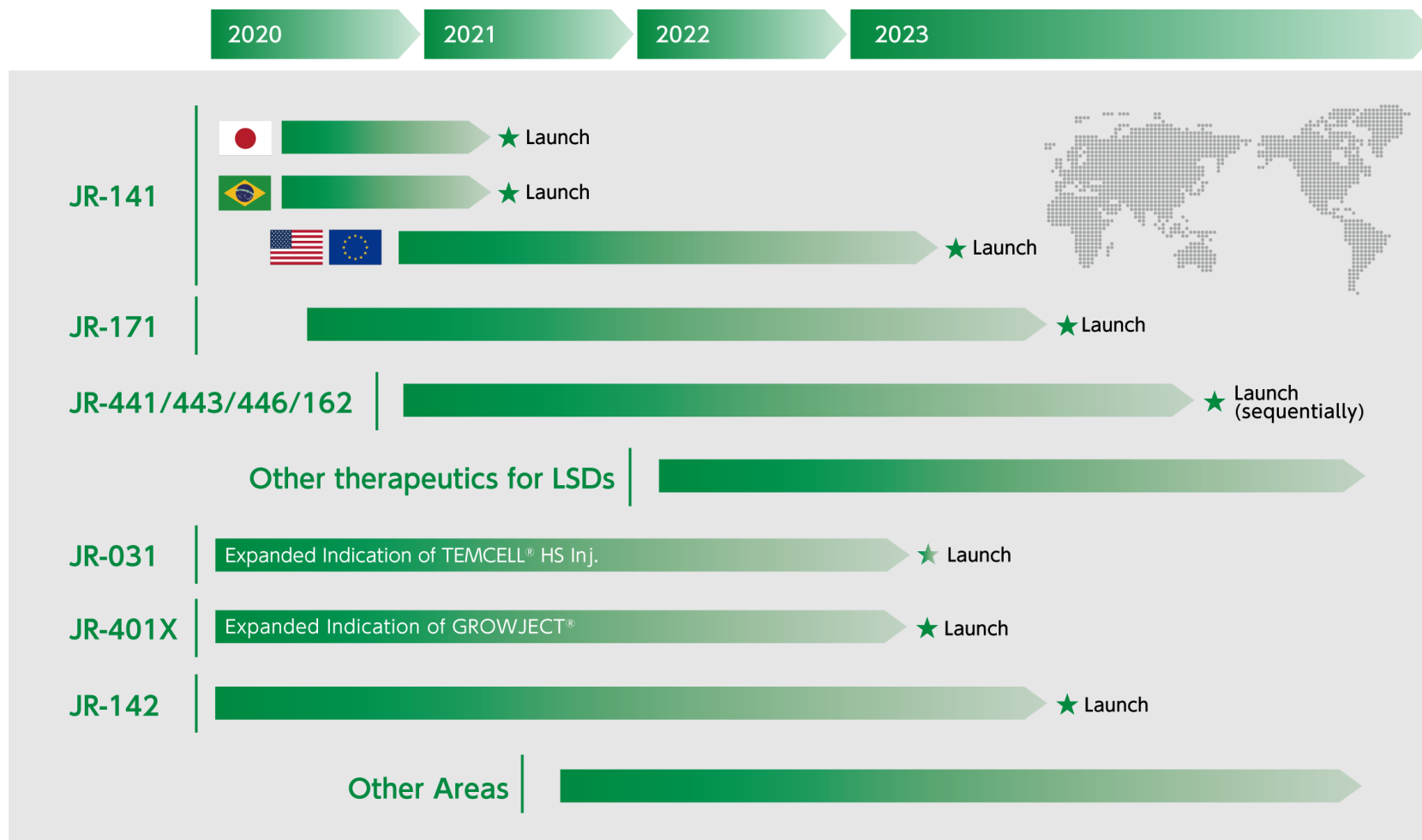
※1 Calculated internally based on the date from MHLW ※2 Actual sales of existing ERT and data from Evaluate Pharma and IQVIA



- Our current antibody-based J-Brain Cargo® technology is more amenable to target proteins rather than small molecules and nucleic acids.
- JCR is now investigating new modalities to enable those moieties to penetrate BBB by expanding the technological possibilities of J-Brain Cargo®, a transferrin receptor mediated drug delivery system. JCR seeks to create next generation of J-Brain Cargo® technologies through combination with other in-house technologies as well as through joint research with partners for constrained peptides and nanomicelles.
- JCR is also now investigating the possibility of gene therapy technology. Our R&D activities may lead to creation of new treatment options to address, for example, central nervous system diseases caused by a lack of essential molecules or genetic mutations.
- In the area of regenerative medicine, JCR is taking on the challenges in creating new treatment options for unmet medical needs leveraging our unique stem cell and recombinant DNA technologies.



**JCR is taking on challenges to create and deliver first-in-class drugs from Japan with our proprietary technologies.**



\* Only in-house R&D activities are listed.

# Midterm Business Plan for 2020-2022

## “REVOLUTION”



### ***Rare Diseases***

*Niemann-Pick disease*

*Metachromatic leukodystrophy*

*Batten disease*

*GM1 gangliosidosis*

*Gaucher disease*

*Hurler/Hurler-Scheie/Scheie syndrome*

*Hunter syndrome*

*Sly syndrome*

*Fucosidosis*

*Sanfilippo syndrome A/B*

*GM2 gangliosidosis*

*Alpha-Mannosidosis*

*Krabbe disease*

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***REVOLUTION***  
*into the Future*

By uniting our “Team JCR” capabilities,  
JCR will pursue our “**REVOLUTION**” in  
quality and quantity for business toward 2030.

# “REVOLUTION”: Important Business Challenges

JCR will be addressing the 6 important business challenges below in anticipation of our full-fledged globalization.

## Top priority business challenge

in anticipation of growing presence of JCR in the rare disease area

### [1] **Qualitative and quantitative reorganization of the quality assurance system**

Furthermore, JCR accelerates agendas listed below as important business challenges in anticipation of a rapid expansion of business in the late 2020s.

For strengthening our foundation for profits:

### [2] **Action for sustainable growth of the sales of our products**

Exploring new therapeutic targets in addition to lysosomal storage diseases:

### [3] **Expansion of basic research activities**

For full-fledged globalization in the near future:

### [4] **Evaluation and implementation of further capital investment for manufacturing and research**

For maximizing business values in the lysosomal storage disease area:

### [5] **Product strategy planning including evidence generation**

For our full-fledged globalization:

### [6] **Transformation of operations and organizations along with human resource development**



# REVOLUTION : GUIDANCE

Sales and Operating income in FY2022

Sales : **¥32~36 billion**

Operating income : **¥7~10 billion**

(To target steady growth with year-to-year rise)

R&D expenditures

Around **20%** of sales

(Beyond 20% could be allocated, if required)

Dividend Ratio

Around **30%**

(Under a stable dividend policy, weighing an anticipation of our stockholders and the balance of financial soundness)

# JCR's Sustainability : Basic Policy during “REVOLUTION”

JCR will commit to development of a sustainable society through our corporate philosophy, “Contributing towards people’s healthcare through pharmaceutical products”.

Basis for  
value creation

Realizing medical care for those living with rare diseases

## Basic Policy for Our Sustainability

JCR will commit to realization of sustainability in tune with the spirit **“No one will be left behind”** mentioned in the SDGs (Sustainable Development Goals) through our activities to address these challenges and to share and return the achievements to our stakeholders focusing on **“Rare Diseases”, “Environment”, “Society” and “Corporate Governance”**.

# JCR's Sustainability : Efforts during “REVOLUTION”

JCR will commit to expansion of sustainability efforts in quality and quantity in order to become a global specialty pharma company in the rare disease arena.

Target | Realization of sustainability through activities based on RD · E · S · G

Direction



## Rare Diseases

- Expansion of basic research for the treatments for rare diseases
- Activities to gain recognition for rare diseases
- Internal enlightenment activities for rare diseases

Contribution  
through our business

Unite the capabilities of “Team JCR” for the “REVOLUTION” of our business in quality and quantity  
**Acceleration of “Realizing medical care for those living with rare diseases”**



# JCR's Sustainability : Efforts during "REVOLUTION"

JCR will commit to expansion of sustainability efforts in quality and quantity in order to become a global specialty pharma company in the rare disease arena.

Target | Realization of sustainability through activities based on RD · E · S · G

Direction



**E**

## Environment

- Activities rooted in the community to reduce environmental burden



**S**

## Society

- Improvement of work environment and cultivation of human resources for "Team JCR"
- Support for rare pediatric diseases and healthcare



**G**

## Corporate Governance

- Enforcement of the system for "stable supply" in anticipation of globalization
- Enforcement of a management system which can make audacious and appropriate decisions
- Implementation of risk management

Contribution  
through our business

Unite the capabilities of "Team JCR" for the "REVOLUTION" of our business in quality and quantity  
**Acceleration of "Realizing medical care for those living with rare diseases"**



Thank you for your attention.



What JCR can do  
for rare diseases