



Midterm Business Plan for FY2023-2027

“Reach Beyond, Together”

May 11, 2023

JCR Pharmaceuticals Co., Ltd.



We have provided society with pharmaceuticals featuring new mechanisms available for the first time anywhere in the world such as TEMCELL[®] and IZCARGO[®]. Our J-Brain Cargo[®] technology, which we have established and focused on developing further, will enable us to provide patients around the world with drugs that can be expected to have significant benefits for diseases that were previously untreatable.

JCR is now one of the few companies in Japan that can operate the entire process of biopharmaceuticals, from research through to manufacturing, and is focusing on further accelerating R&D for rare diseases, particularly Lysosomal Storage Diseases. We will also greatly expand the scope of R&D while cooperating with partners. Through developments such as innovative protein drugs and gene therapy, we will deliver "medicines that can only be made by JCR" for as many patients as possible and their families.

During the upcoming second foundation, we will make aggressive investments in R&D in order to continue generating new value with pharmaceuticals that have been developed in Japan for the first time in the world.

Shin Ashida

Representative Director, Chairman,
President, CEO and COO



- 1. Our Credo**
- 2. Positioning of Midterm Business Plan for FY2023-2027**
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“REVOLUTION”
- 4. Midterm Business Plan for FY2023-2027**
“Reach Beyond, Together”
 - Five Initiatives**
 - Sustainability Initiatives**
 - Toward 2027 and Beyond**



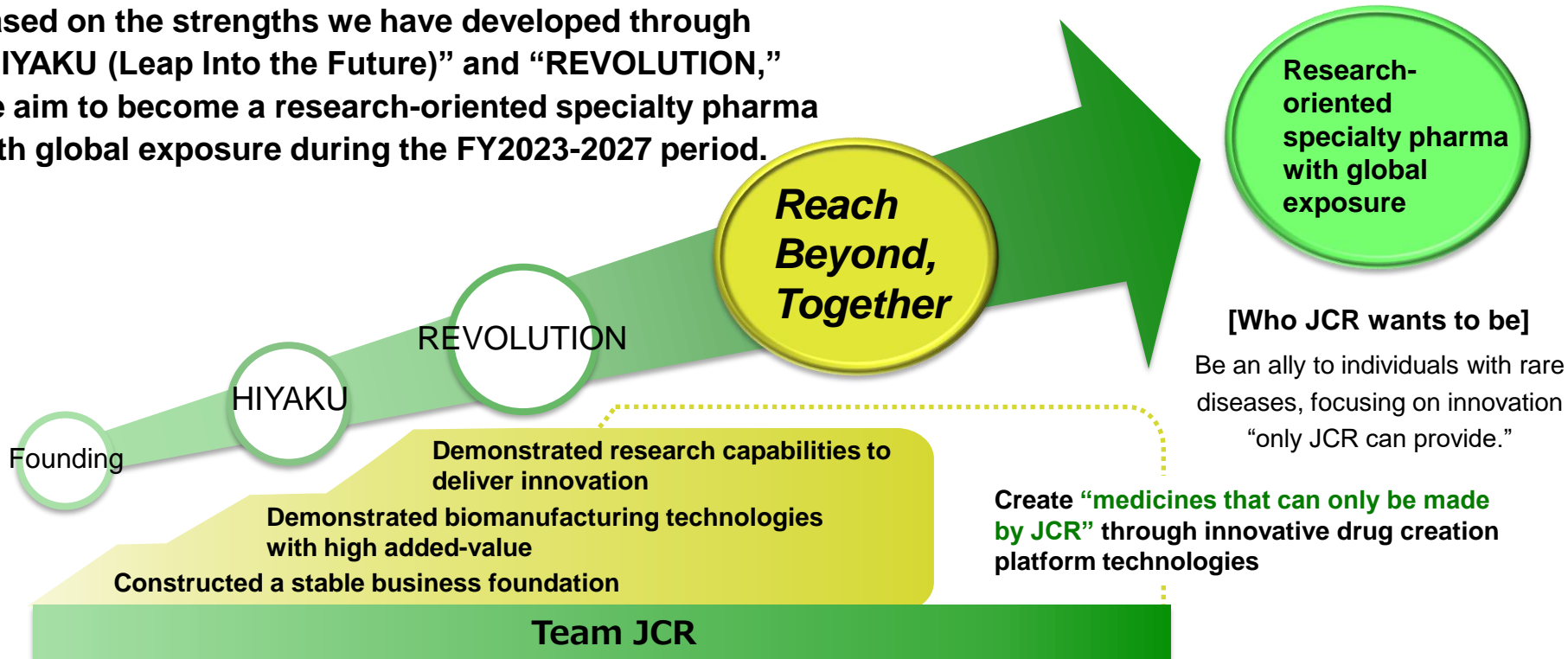
Having built foundational “R&D” and “Manufacturing” capabilities, we can deliver value only JCR can provide to patients with rare diseases and their families.



Corporate Philosophy

Contributing towards people's healthcare through pharmaceutical products.

Based on the strengths we have developed through "HIYAKU (Leap Into the Future)" and "REVOLUTION," we aim to become a research-oriented specialty pharma with global exposure during the FY2023-2027 period.



Summary of Midterm Business Plan for FY2020-2022 “REVOLUTION”

变革

REVOLUTION
into the Future

Summary of “REVOLUTION”: Our Growth

JCR has achieved continuous growth through strategic investment to realize new added value for society, based on a robust management base.

	HIYAKU		REVOLUTION			
	FY2015	FY2019	1st year FY2020	2nd year FY2021	Last year FY2022	Guidance (Target for FY2022)
Net sales (¥ billion)	17.4	24.7	30.0	51.0	34.3	32.0 to 36.0
Operating income (¥ billion)	2.1	3.2	8.2	19.9	4.9	7.0 to 10.0
R&D expenditures (¥ billion) (to sales)	3.3 (19.2%)	5.9 (24.2%)	5.3 (17.8%)	7.1 (14.0%)	8.8 (25.6%)	Approx. 20%
Capital investment (¥ billion)	1.2	5.2	3.9	10.6	8.0	-
Employees (Consolidated)	526	667	732	817	879	-
Dividend payout ratio	39.2%	36.8%	21.5%	18.8%	65.9%	Approx. 30%

Review of “REVOLUTION”: Six Important Accomplishments

Key imperatives	Accomplishments
Qualitative and quantitative reorganization of the quality assurance system	Remodeled QA system from research to manufacturing—Analytical R&D center Improved data integrity—Introduction of LIMS
Action for sustainable growth of the sales of our products	GROWJECT®—Developed Melon Nikki™ app for improving treatment adherence Developed fully-automated electric injector and novel device development
Expansion of basic research activities	Generated 17 drug candidates for Lysosomal Storage Diseases Applied J-Brain Cargo® to various modalities—partnered with multiple companies AlliedCel, a joint venture to realize the social implementation of regenerative medicine and cell therapy
Evaluation and implementation of further capital investment for manufacturing and research	Expanded DS/DP capacity, enabling multiple developments in parallel – Expanded research center, Kobe Science Park Center – Established collaboration with Mycenax Biotech Inc. through equity participation
Product strategy planning including evidence generation	Communicated the value of J-Brain Cargo® technology via conferences and publications Sales forces specialized in pediatric field Transferred marketing rights for Agalsidase Beta BS to Sumitomo Pharma
Transformation of operations and organizations along with human resource development	Optimized organizational efficiency, started global development, and created international footprints. Established JCR Academy to nurture next-generation leaders with global perspective Implemented state-of-the-art IT infrastructure to improve productivity Reformed workplace policies

Achievements


Realization of J-Brain Cargo® technology

- Entering development stage of JR-443, JR-446, JR-479, JR-471
- **Launch of IZCARGO®**, the world's first-ever BBB penetrating type ERT, in Japan
- Agreement with **Takeda** for joint development and business development of JR-141
- Agreement with **Takeda** for joint R&D and license of gene therapy, milestone achievement for gene therapy
- Agreement with **Medipal Holdings** on Ultra Four project (four ultra-rare lysosomal storage diseases)
- Out-licensing of J-Brain Cargo® to **Alexion, AstraZeneca Rare Disease (Alexion), Angelini, and Sumitomo Pharma**

Strategic CAPEX and demonstration of prominent biomanufacturing capability

- Investment in Mycenax, CDMO in Taiwan
- Completion of Kobe Science Park Center
- **Completion of the AstraZeneca COVID-19 vaccine drug substance production**

Foundation for global expansion

- 
- **JCR Europe (2022)**
 - **JCR USA (2018)**
 - **JCR DO BRASIL (2020)**
 - Global distribution hub in Luxembourg

Significance for the future

- Applicability of J-Brain Cargo® to various disease areas
- Foundation of licensing revenue from partnering strategy
- Supply chain construction towards global standard manufacturing
- Realization of our globalization based on subsidiaries

Midterm Business Plan for FY2023-2027

Reach Beyond, Together

Five Initiatives

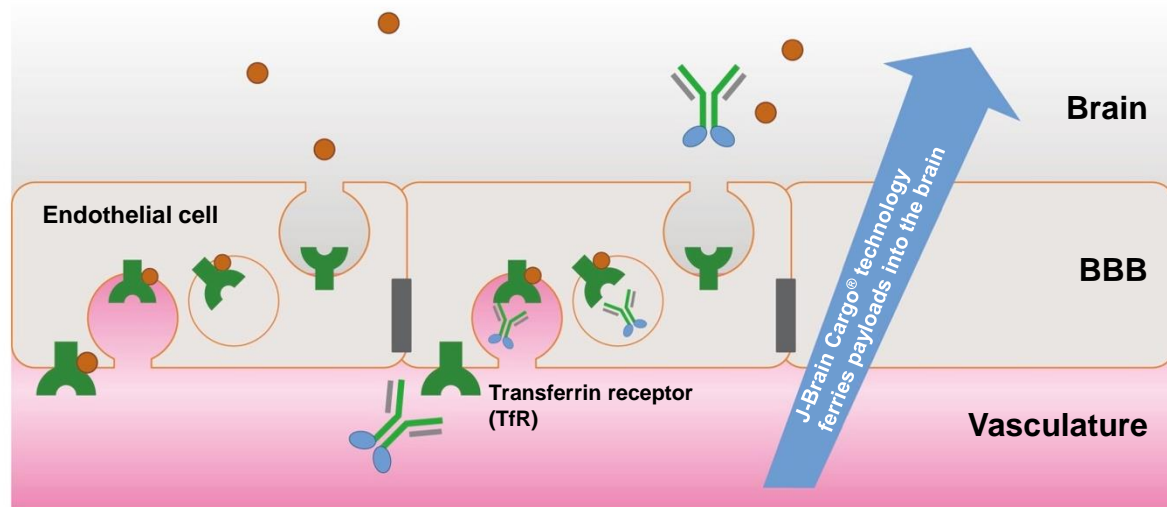
1 Creation of innovative core technologies

2 Demonstrating global standard production capacity

3 Expansion of global quality assurance system
in terms of quality and quantity

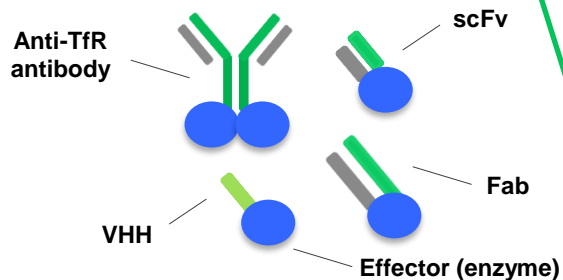
4 Early launch of products for rare diseases

5 Human resource development
to support growth



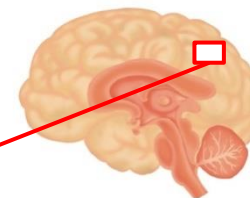
● Transferrin

J-Brain Cargo® -applied therapeutics



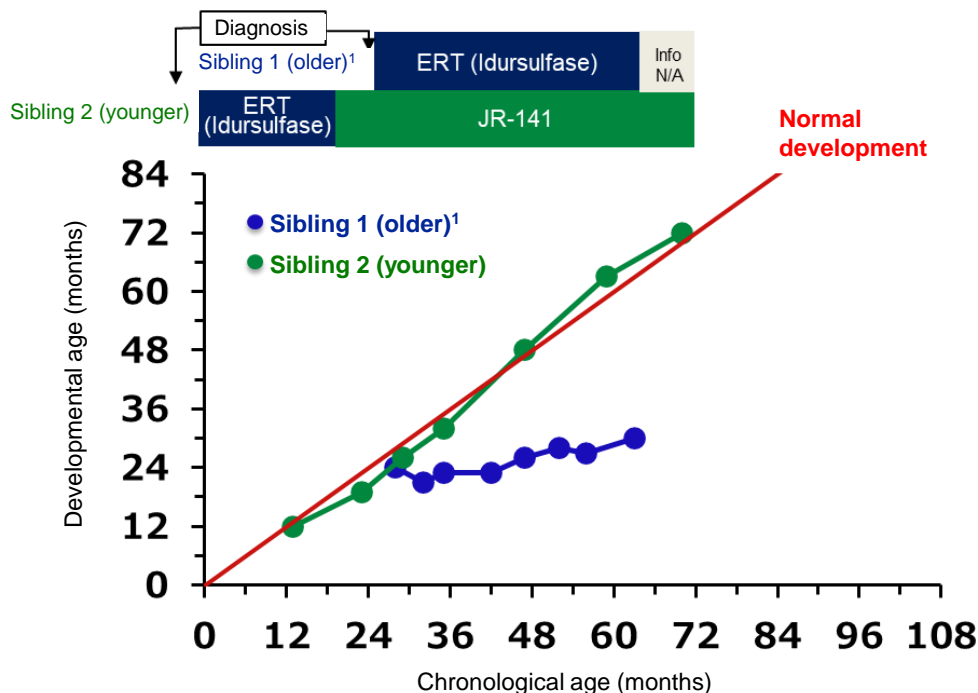
Selecting J-Brain Cargo® molecules best suited for unique effector properties

- Plentiful J-Brain Cargo® variations
- Through the application of protein engineering technology:
 - Optimized binding affinity with receptors
 - Creation of biopharmaceuticals that are difficult to produce



IZCARGO®, world's first drug proven to penetrate the BBB to deliver ERT to CNS. Launched in Japan in 2021.

Case report in a sibling case of MPS II with identical genetic mutation



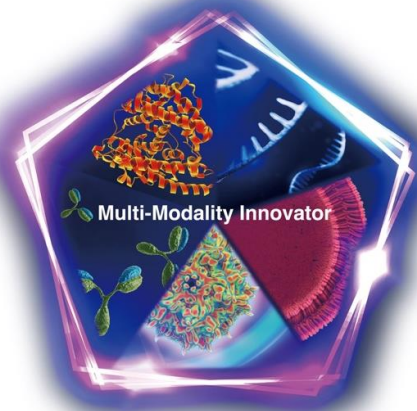
For Sibling 2, normal development was confirmed over 72 months old (as of November 2022), following treatment for pabinafusp alfa (JR-141) (planning to obtain long-term data in the future as well)

J-Brain Cargo® technology is a platform technology that delivers drugs necessary for the central nervous system across the blood-brain barrier, and is expected to be applied to various diseases

1. Based on Tomita K et al., JIMD Reports, 2021, 1-6

Cutting-edge drug development following J-Brain Cargo® technology

The J-Brain Cargo® Platform reaches beyond the CNS



J-Brain Cargo®

J-XXX Cargo

J-YYY Cargo

⋮

Target organ



Brain



Eye



Skeletal muscle



Cartilage



Other

Typical examples of target diseases

Lysosomal Storage Disease

Neurodegenerative Disease

Ocular Disease

Bone Disease

Muscular Disease



Approval of IZCARGO®(Japan)

Gene therapy Partnership with Takeda



JBC partnership with Alexion, Angelini



2020

2021

2022

2023

IZCARGO® partnership with Takeda



Ultra-rare diseases partnership with MEDIPAL



JBC partnership with Sumitomo Pharma (2015) Sumitomo Pharma

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Five Initiatives

1

Creation of innovative core technologies

2

Demonstrating global standard production capacity

3

**Expansion of global quality assurance system
in terms of quality and quantity**

4

Early launch of products for rare diseases

5

Human resource development
to support growth

Strength in “MONOZUKURI” (Manufacturing)



Seishin Plant



Kobe Plant



Research Institute



Murotani Plant



Kobe API Plant



Bioresearch Center



Clinical Trial Material Manufacturing Center and Cell Processing Center (CPC)



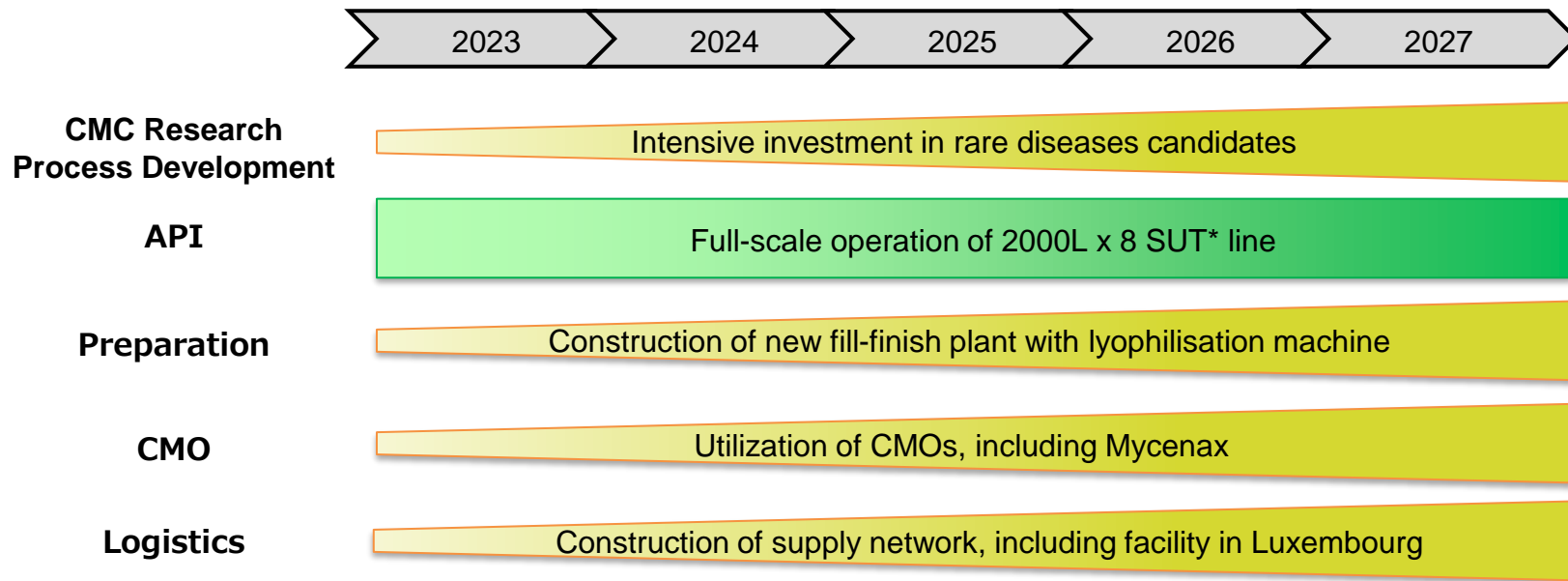
New formulation building
(To be completed in 2027)

Kobe Science Park Center
(Completed in 2022)

- Our sites are operating at full capacity.
- More than 15 years of manufacturing experience with single-use technologies
- Integrated quality control system from research to product
- Quality assurance system dedicated to biopharmaceuticals
- Approximately 400 highly skilled employees
- Close cross-sectoral collaboration between physically adjacent research and production in Kobe area

Site	Production Capacity
Murotani Plant 1	2x 400L; 2x 80L
Murotani Plant 2	2x 2,000L; 2x 200L (Single-use)
Clinical Trial Material Center	1x500L, 1x200L (Single-use)
Kobe API Plant	2x 2,000L; 2x 200L (Single-use)
Kobe Plant	Filling and packaging
Seishin Plant and CPC	Regenerative medical products
Kobe Science Park Center	4x 2,000L, 4x 200L (Single-use)

- Over \$300m CAPEX and investment in CDMOs, leading to supply expansion.
- Constructing a resilient global supply chain for emergencies.



Existing drugs

Domestic market

- Growth hormone
- Biosimilars
- TEMCELL® HS Inj.



Initiatives for stable supply

- Sophisticated procedures and robust quality control
- Securing raw materials
- Securing appropriate benefits

Drugs for Rare Diseases Vaccines

Global strategic items

- J-Brain Cargo® development products: 10+
- Gene therapy products
- Vaccines (upon government request)






Initiatives for frontiers

- Manufacturing with new technologies
- GMP audit by regulatory authorities from outside Japan
- On-track development
- Small lot size

Five Initiatives

- 1 Creation of innovative core technologies
- 2 Demonstrating global standard production capacity
- 3 Expansion of global quality assurance system in terms of quality and quantity
- 4 Early launch of products for rare diseases**
- 5 Human resource development to support growth

Basic research	Non-clinical	Clinical (Phase I/II)	Clinical (Phase III)	Review period	Approved
JR-194 (Batten's disease type I)	JR-441 MPS type III A (Sanfilippo A syndrome)	JR-171  MPS type I (Hurler syndrome, etc.)	JR-141  MPS type II (Hunter syndrome)		JR-141  MPS type II (Hunter Syndrome)
Krabbe disease	JR-446 MPS type III B (Sanfilippo B syndrome)				
GM1 gangliosidosis					
CLN2 (Batten's disease type II)	JR-162 Pompe disease				
Gaucher disease	JR-443 MPS type VII (Sly syndrome)				
α-Mannosidosis					
Niemann-Pick	JR-479 GM2 gangliosidosis (Tay-Sachs, Sandhoff disease)				
Metachromatic leukodystrophy					
Galactosialidosis	JR-471 Fucosidosis				

Aiming to begin clinical trials for five items
(Candidates)
JR-441, JR-446, JR-479, JR-471, JR-194, Krabbe disease

Five Initiatives

- 1 Creation of innovative core technologies
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Human Resource Strategy

Continue investment in human capital, and establish a human resource strategy that helps to improve corporate value

Construction of a dynamic human resource portfolio that will help to accomplish the strategy

- Assessing the current status of our human resource portfolio
- Ensuring diversity through mid-career recruitment, producing synergetic effects with pool of existing human resources
- Creating the optimal human resource portfolio

Development and organizational permeation of diversity & inclusion

- Deepening “R&D” and “Monozukuri” (manufacturing) through inclusion of knowledge and experiential value possessed by professional human resource with diverse attributes
- Management and monitoring of inclusion

Activation of individuals and organizations Promotion of improvements to engagement

- Improving engagement by proactively transmitting JCR’s vision both in and outside the company
- Support for employee growth, ambition throughout the entire organization through establishing an environment, enhancing energy

Build a human resource management frame as a framework to realize the image of who we want our people to become

- Designing a mechanism for performance evaluation centered on “contribution”
- Setting a perspective for evaluating the challenges of advanced issues

- Job responsibilities and various status settings that allow for medium-term career development
- Introduction of diverse role frames that take the organizational mission into account



- Wage system emphasizing “Role and Contribution,” upgraded from a wage system based on “Seniority and Position”
- Wage system considering the life-work balance of employees

- Introduction of mechanisms to promote career autonomy
- Deepen compliance program
- Strengthen programs reflecting a diversity management perspective as a result of globalization

Ideal state of mind

Achieve sustainability through business activities based on RD-E-S-G



Rare Diseases

- Development of drugs for ultra-rare diseases and construction of new business models
- Efforts to improve access to medicines (Named Patient Supply) to deliver innovative medicines to people around the world



Environment

- Investigation and implementation of measures to reduce environmental impact through business activities
- Promote environmentally friendly procurement on the premise of ensuring the quality and stable supply of pharmaceuticals



Society

- Address the challenge of unmet medical needs by creating platform technologies
- Develop human resources so that each member of "Team JCR" can shine in their own station



Corporate Governance

- Pursue governance in line with the company's growth
- Ensure thorough compliance and strengthen risk management



Initiatives for Rare Diseases



JCR aims to realize medical care that leaves no one behind through our business activities.

Our initiatives for LSDs

To all patients around the world

Rare Diseases

Ultra-Rare Diseases

JR-162	Pompe	Gaucher	Fabry disease	
JR-171	MPS I (Hurler)	JR-441	MPS IIIA (Sanfilippo A)	
JR-141	MPS II (Hunter)	JR-479	GM2 gangliosidosis	GM1 gangliosidosis
JR-446	MPS IIIB (Sanfilippo B)	MLD		
	Krabbe disease	CLN2 (Batten disease type II)	Niemann-Pick	
JR-194	CLN1 (Batten disease type I)			
JR-471	Fucosidosis	JR-443	MPS VII (Sly)	
	Galactosialidosis	α -Mannosidosis		

Partnership with Takeda



Out-licensed to Medipal



Tackling the Challenge of Realizing a Sustainable Society



Realization of sustainable medical care	Growth of people and organizations	Tackling the challenge of solving social issues																			
<p>We will continue to maximize the value created through our business activities and return it to all people, especially patients. We will continue our efforts to respond to the unmet medical needs of patients and their families with innovative drug discovery centered on research and development and manufacturing.</p>	<p>We will build an organization in which each employee can grow with a career outlook and return new value to society. We value our unique corporate culture and core values, which are not constrained by fixed ideas, and strive to enhance the working environment that fosters the abundant creativity of employees and realizes gender equality.</p>	<p>We will realize business activities that take the natural environment into consideration. We will strive to raise awareness of the effective use of limited resources so that each and every employee can take the initiative to act.</p>																			
Initiatives																					
<p>Continued support of researchers Contributions to the Kyoto University Honjo Tasuku Fund and the Cancer Immunotherapy Research Fund (allotment of treasury shares)</p>	<p>Training of global workers</p> <ul style="list-style-type: none"> • Enhancement of training systems to support employee growth • Develop next-generation global leaders (JCR Academy) 	<p>Initiatives for Carbon Neutrality</p> <ul style="list-style-type: none"> • Installation of solar power generation systems (Research Laboratories, Kobe Science Park Center and building at HQ) • Reduction of waste (reuse of wastepaper and waste plastics, which are difficult to recycle as biomass boiler fuel) • Adoption of rental uniforms made from recycled materials • Switch to LED lighting • Shift business and other company vehicles to hybrid cars and electric/hydrogen vehicles 																			
<p>Development of medical researchers capable of playing an active role on a global scale Support for the International Foundation for Medical Research</p>	<p>Diversity and inclusion</p> <ul style="list-style-type: none"> • Creating an environment where employees can thrive regardless of gender • Promotion of employment of persons with disabilities • Promotion of diverse work styles including telecommuting Turnover rate (permanent employees): 2.5% in FY2019 / 2.8% in FY2022 • "2018 (3rd) Hyogo Women's Active Participation Company" Promote initiatives to develop in-house childcare centers Encouragement of male participation in childcare • Obtained "Eruboshi (second stage)" certification • Received the "Corporate Award for Balancing Work and Life" 																				
<p>Support for pediatric medical care and insurance medical care Support for the Mother and Child Health Incentive Award</p>																					
<p>Medical, welfare and nursing care support Support for the Medical Service Award</p>																					
<p>Realization of medical care for patients and their families Continuous support for the "Momiji no Ie" medical short-term admission facility</p>																					
<p>Initiatives to contribute to global health Support for the activities of the non-profit foundation Global Foundation for Life Sciences</p>																					
<table border="1"> <thead> <tr> <th></th> <th>FY2015</th> <th>FY2019</th> <th>FY2022</th> </tr> </thead> <tbody> <tr> <td>Percentage of female employees</td> <td>33.2%</td> <td>37.2%</td> <td>40.6%</td> </tr> <tr> <td>Percentage of female managers</td> <td>6.2%</td> <td>10.7%</td> <td>12.5%</td> </tr> <tr> <td>Percentage of female new graduates</td> <td>27.3%</td> <td>31.8%</td> <td>59.1%</td> </tr> <tr> <td>Percentage of acquisition of childcare leave (male)</td> <td>0.0%</td> <td>14.2%</td> <td>66.7%</td> </tr> </tbody> </table>			FY2015	FY2019	FY2022	Percentage of female employees	33.2%	37.2%	40.6%	Percentage of female managers	6.2%	10.7%	12.5%	Percentage of female new graduates	27.3%	31.8%	59.1%	Percentage of acquisition of childcare leave (male)	0.0%	14.2%	66.7%
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Since its founding in 1975, JCR has continued to grow by developing its unique “R&D” and “Monozukuri (manufacturing)”. The creation of J-Brain Cargo® technology, which is one of its monumental achievements, can potentially be applied to not only the field of rare diseases but the treatment of various diseases as well.

Additionally, we believe that it is JCR’s mission to create innovative platform technologies following J-Brain Cargo® technology, deliver the drugs generated through those technologies to patients and their families around the world, and contribute to treatments.

In order to carry out this mission, rather than establish quantitative guidance in this Midterm Business Plan, we will determinedly invest in the opportunities for growth through advancements in R&D. Then, we will aim to maximize value for patients and their families, JCR employees and shareholders through the creation of new innovation.

The second foundation marks the beginning of a new challenge toward growth.

– For patients and their families –

Reach Beyond, Together



Reach Beyond, Together

Reach Beyond, Together



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.