FY2021 Second-Half Results Briefing Session - Business Highlights -

May 12, 2022

JCR Pharmaceuticals Co., Ltd

【Securities code】4552, PRIME. TSE

Agenda

- R&D Highlights
- R&D Updates
- Vaccine Stock Production Business

- Interim Summary of Midterm Business Plan “REVOLUTION”

Research and Development Highlights (Sep 2021 – Apr 2022)

2021

◆Sep. Entered into an exclusive collaboration and commercialization agreement for **JR-141** (pabinafusp alfa) **with Takeda** in certain regions

◆Oct. EMA grants **PRIME DESIGNATION** to **JR-141** (pabinafusp alfa).

2022

◆Jan. EMA grants Orphan Designation to **JR-441** for Sanfilippo type A syndrome

◆Feb. **IZCARGO®** granted **New Treatment Award** by WORLD*Symposium*™ 2022.

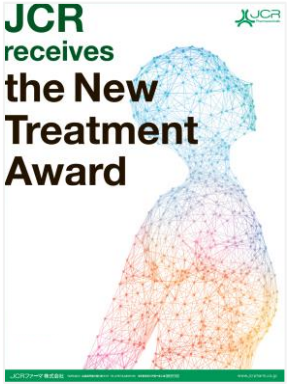
◆Feb. **First patient dosed in JR-141** (pabinafusp alfa) Global Phase 3 Clinical Trial

◆Mar. **JR-479** for GM2 gangliosidosis enters **Development**

◆Mar. **Exclusive license and collaboration agreement** concluded with Takeda to develop **gene therapies using J-Brain Cargo® technology** for lysosomal storage disorders

◆Mar. R&D Meeting held for institutional investors and analysts

◆Apr. Teijin and JCR research on JTR-161 dental pulp stem cells terminated



Development Portfolio Overview

★ : progress in development stage since November 2021

	Indication	Preclinical	Clinical Trials	Filed	Approved	Remarks/ Time to next value inflection point
JR-141	MPS type II (Hunter Syndrome)	Approved / Filed / Phase 3				• J-Brain Cargo® • FY2025～ (Approval in US, EU)
JR-171	MPS type I (Hurler Syndrome etc.)	Phase 1/2				• J-Brain Cargo® • FY2023 (pivotal trial)
JR-162	Pompe disease	Preclinical				• J-Brain Cargo®
JR-441	MPS type IIIA (Sanfilippo A Syndrome)	Preclinical				• J-Brain Cargo® • FY2023 (phase 1/2)
JR-443	MPS type VII (Sly Syndrome)	Preclinical				• J-Brain Cargo®
JR-446	MPS type IIIB (Sanfilippo B Syndrome)	Preclinical				• J-Brain Cargo® • FY2023 (phase 1/2)
★ **JR-479**	GM2 Gangliosidosis (Sandhoff, Tay-sachs disease)	Preclinical				• J-Brain Cargo® • ~FY2025 (phase 1)
JR-401X	SHOX deficiency	Phase 3				• Expanded indication of GROWJECT® • FY2023 approval in Japan
JR-142	Pediatric growth hormone deficiency	Phase 2				• Recombinant long-acting GH • FY2023 (phase 3)
JR-031HIE	Hypoxic ischemic encephalopathy in neonates	Phase 1/2				• Expanded indication of TEMCELL®HS Inj.

MPS: Mucopolysaccharidosis ERT: Enzyme replacement therapy

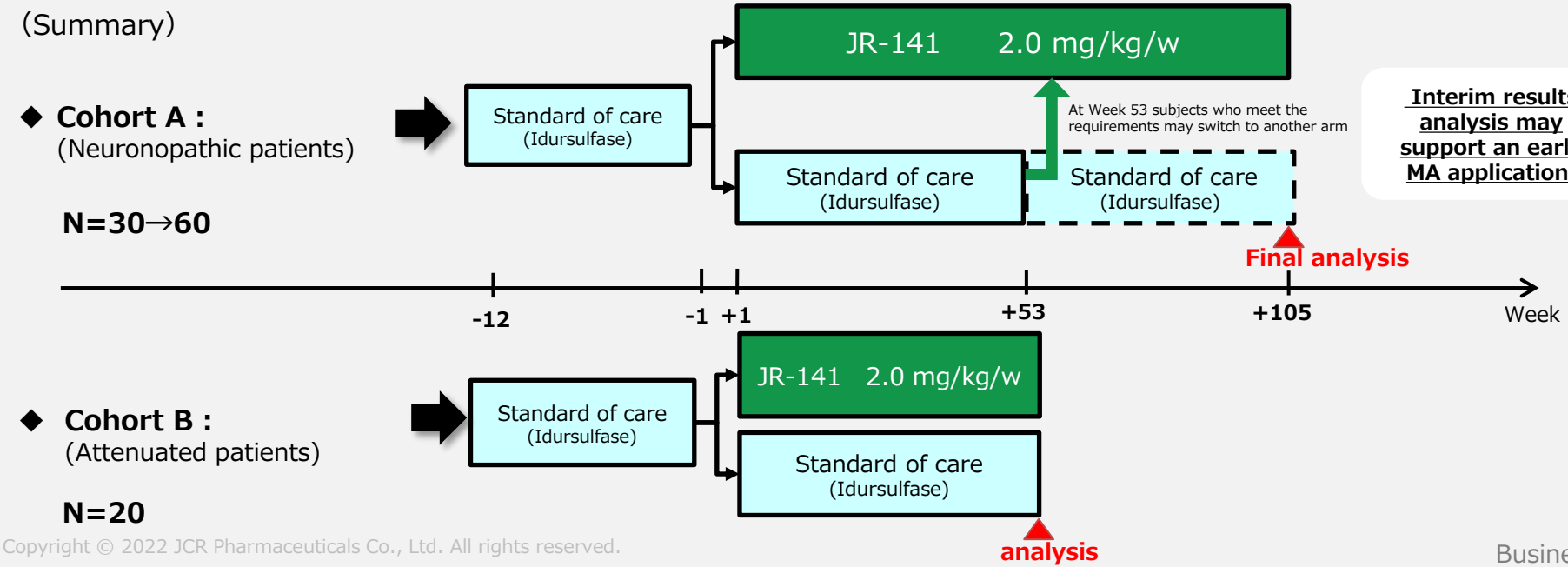
Progress on JR-141 (JR-141-GS31)

JR-141

IZCARGO® (Brand name in Japan)
pabinafusp alfa: BBB-penetrating iduronate-2-sulfatase (rDNA origin)

- **Sep. 2021: Entered into an exclusive collaboration and commercialization agreement for JR-141 with Takeda in certain regions**
- **Oct. 2021: PRIME Designation from EMA**
- **Feb. 2022: First Patient Dosed in global phase 3 study**
- **Number of subjects in cohort A changed from 30 to 60**

(Summary)

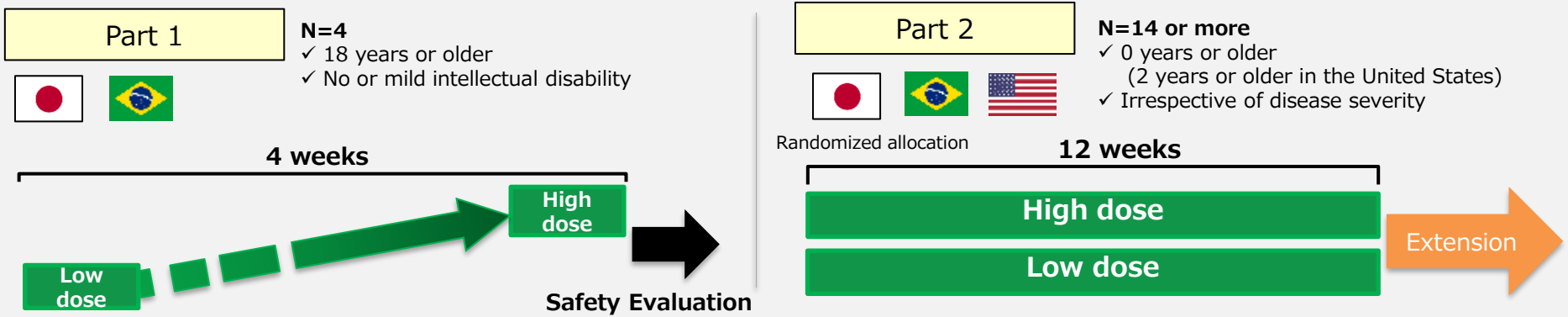


Progress on JR-171

JR-171 lepunafusp alfa: BBB-penetrating α-L-iduronidase (rDNA origin)

Summary of Global Phase 1/2 Clinical Trial (JR-171-101)

◆ **May 2022: Completed patient recruitment of Part 2**



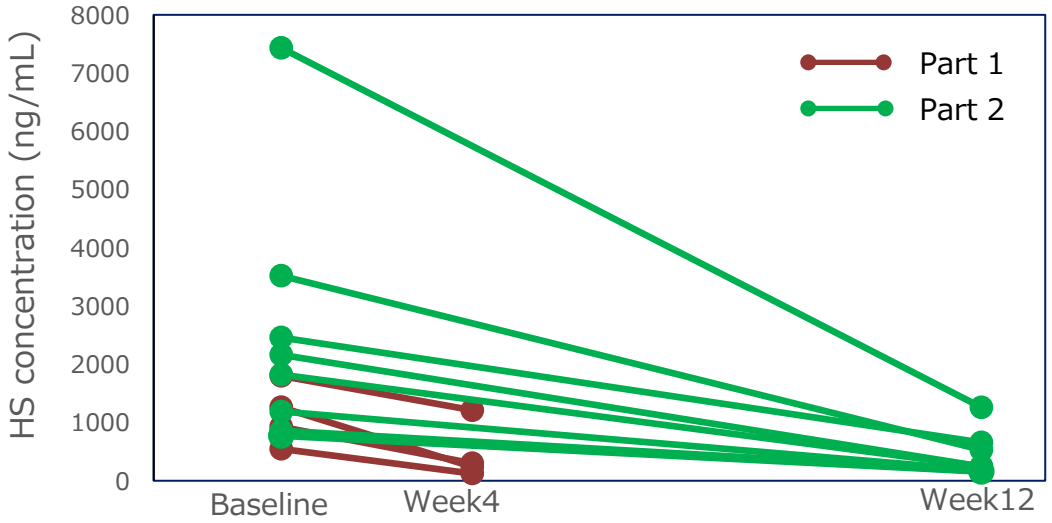
	Part 1	Part 2
Primary endpoint	Safety	
Secondary and exploratory endpoints	・Plasma drug concentration, pharmacokinetic parameters ・Exploratory efficacy for central nervous system and systemic symptoms	
Geography	Japan・Brazil	Japan・Brazil・USA
Clinical trials identifier	clinicaltrials.gov NCT04227600	

Progress on JR-171

JR-171 lepunafusp alfa: BBB-penetrating α-L-iduronidase (rDNA origin)

Phase 1/2 Global Clinical Trial（JR-171-101）:

Change in CSF Heparan sulfate (HS) Concentrations as surrogate for substrate reduction in the CNS



CSF HS concentrations decreased in all subjects

CSF: Cerebrospinal fluid

Initiation of Development of JR-479

JR-479 (BBB)-penetrating β-Hexosaminidase A

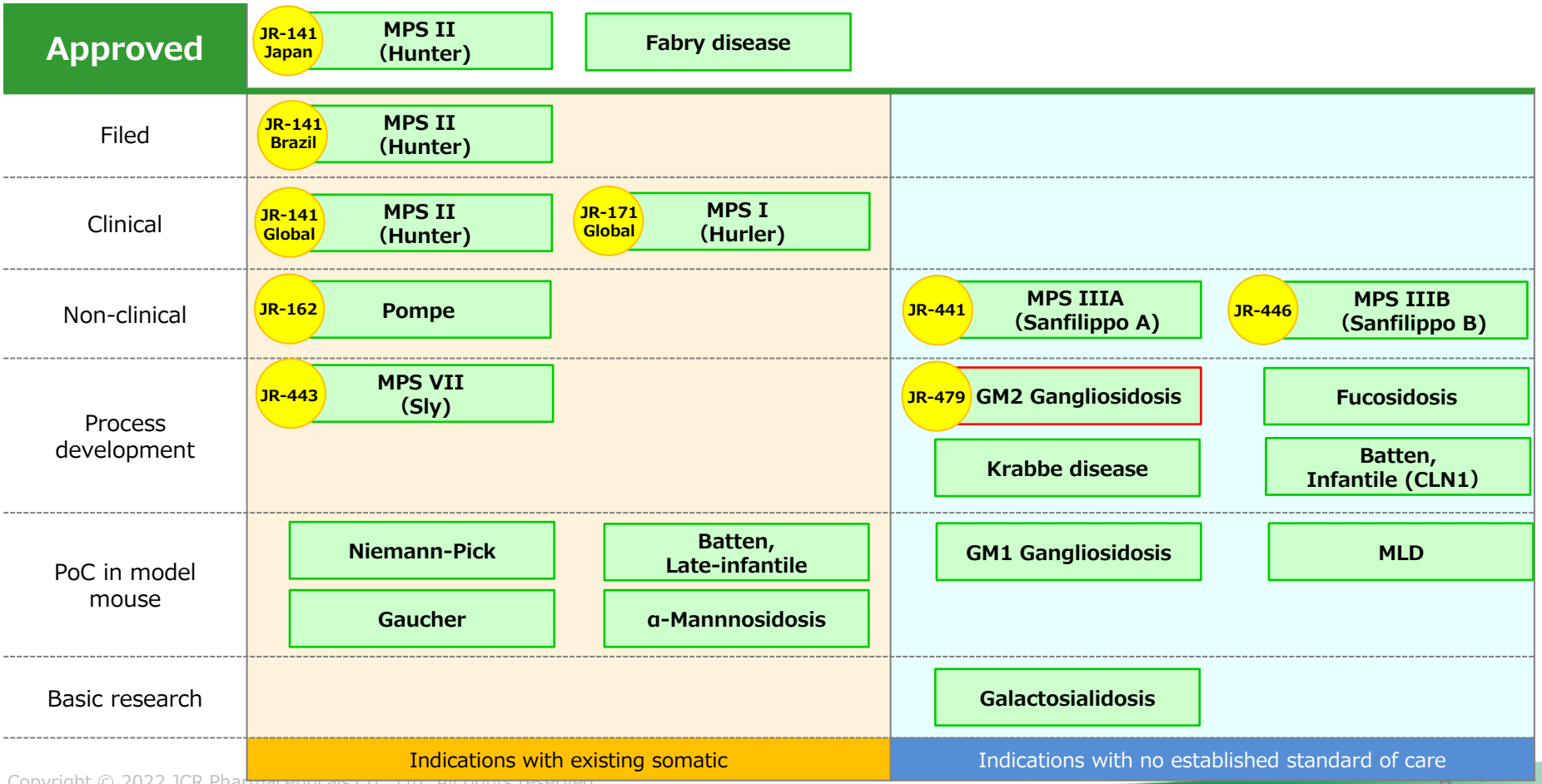
Indication :	**GM2 gangliosidosis** ├ **Tay-Sachs disease** : Deficiency in α-subunit of β-Hexosaminidase └ **Sandhoff disease** : Deficiency in β-subunit of β-Hexosaminidase
Frequency*1 :	Tay-Sachs : 1/100,000～300,000 life births　Sandhoff : Less than Tay-Sachs disease　*1 Internal analysis
Disease overview :	GM2 gangliosidosis is an autosomal recessive LSD caused by a deficiency in the GM2 ganglioside-metabolizing enzyme β-Hexosaminidase A. GM2 ganglioside is abundant in the brain, and GM2 gangliosidosis gives rise to progressive central nervous system (CNS) symptoms. It is difficult to distinguish between Tay-Sachs and Sandhoff disease by clinical symptoms.　LSD: Lysosomal Storage Disease

Type	Age at onset	Clinical symptoms
Classical type (infantile)	3 to 5 months	• Psychomotor developmental delay, regression, visual impairment, hearing impairment, seizures, etc. • Usually leads to death by 3 years of age
Subacute type (juvenile)	2 to 10 years of age	• Similar to infantile type, but slightly milder. Progressive ataxia, regression, convulsions, etc. • Leads to death between 5 and 15 years of age
Late onset	20 to early 30 years of age	• Mild intellectual disability, but characterized by ataxia and progressive neurological symptoms

Animal studies demonstrated the brain delivery of JR-479 and subsequent reduction in causative substrates.
Phase 1/2 study is planned to initiate by 2025.

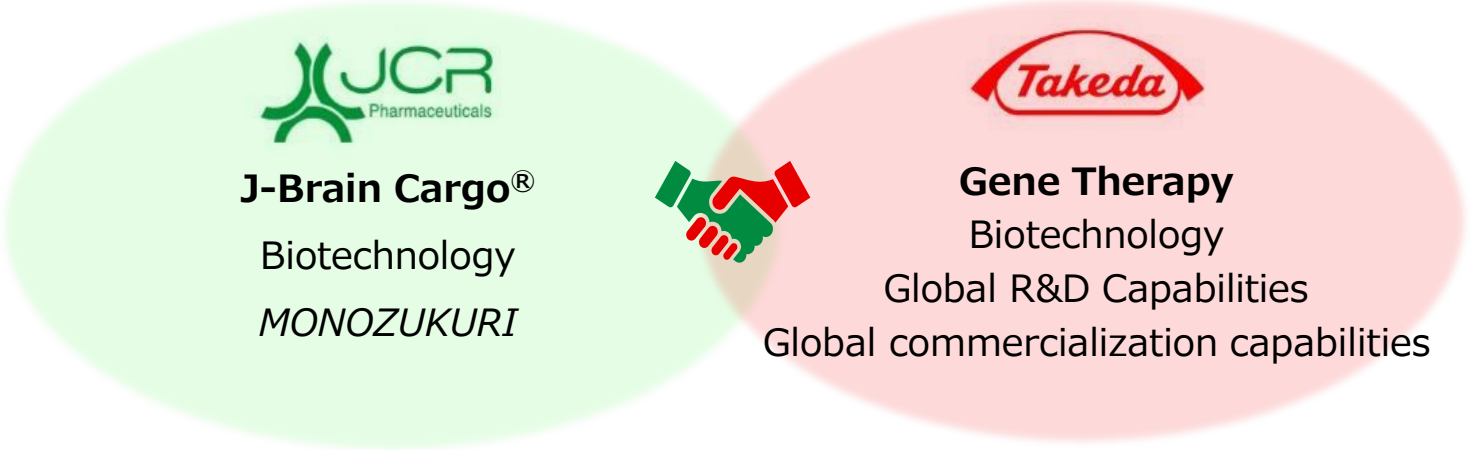
JCR's LSD Pipeline comprises 18 Programs

: progress in development stage since November 2021



Gene Therapy Update

- **Mar. 28, 2022: JCR enters into an exclusive license and collaboration agreement with Takeda to develop gene therapies using J-Brain Cargo® platform.**
  - Initial focus: Lysosomal Storage Diseases
  - Option: Expansion into additional rare diseases and other indications



While keeping our core strengths, focus on developing the next J-Brain Cargo® assets and game-changing medicines

Update on Vaccine Production Business

- **All orders under the Dec. 30, 2020 contract have been fulfilled.**
  - ・Sales recorded based on shipments. (Partial sales will be recorded in FY2022).
  - ・No lot failures demonstrates strength in the manufacturing field.
- **A new plant is under construction in the Kobe Science Park under the Ministry of Health, Labour and Welfare's Sponsorship.**
  - ・Estimated completion of construction : October 2022

Image of "Kobe Science Park Plant"

Agenda

- R&D Highlights
- R&D Updates
- Vaccine Stock Production Business

- Interim Summary of Midterm Business Plan “REVOLUTION”

“REVOLUTION” : Important Business Challenges

JCR will address 6 important business challenges 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 will accelerate agendas listed below as important business challenges in anticipation of a rapid expansion of business in the late 2020s.

For strengthening of 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

Midterm Business Plan “REVOLUTION” : Interim Summary

Qualitative and quantitative reorganization of the quality assurance system	Quality assurance system updated −Newly established the Analytical R&D Center −Started construction of a quality testing building (scheduled for completion in FY2022)
Action for sustainable growth of the sales of our products	Strengthening of GROWJECT®'s sales base −Launch smartphone apps for electric devices and development of new devices Market penetration of IZCARGO® beyond expectations
Expansion of basic research activities	Steady progress in development of LSD products Expansion into various modalities via application of J-Brain Cargo® technology
Evaluation and implementation of further capital investment for manufacturing and research	Expansion of API and formulation manufacturing capacity to enable parallel development of multiple products −Acquired land for construction of a new plant. (47,000m^2)
Product strategy planning including evidence generation	Obtaining long-term clinical data for IZCARGO® Established of an organization dedicated to LSD
Transformation of operations and organization along with human resource development	Reorganized into a functional and efficient organization Development of next-generation of global leaders Expansion of IT infrastructure to improve productivity and reform work styles

Midterm Business Plan “REVOLUTION” : GUIDANCE

- Although the quantitative guidance set forth in the “REVOLUTION” was achieved in FY2021, we will continue to work on the Six Important Business Challenges and accelerate REVOLUTION with the aim of maximizing value in our global business.

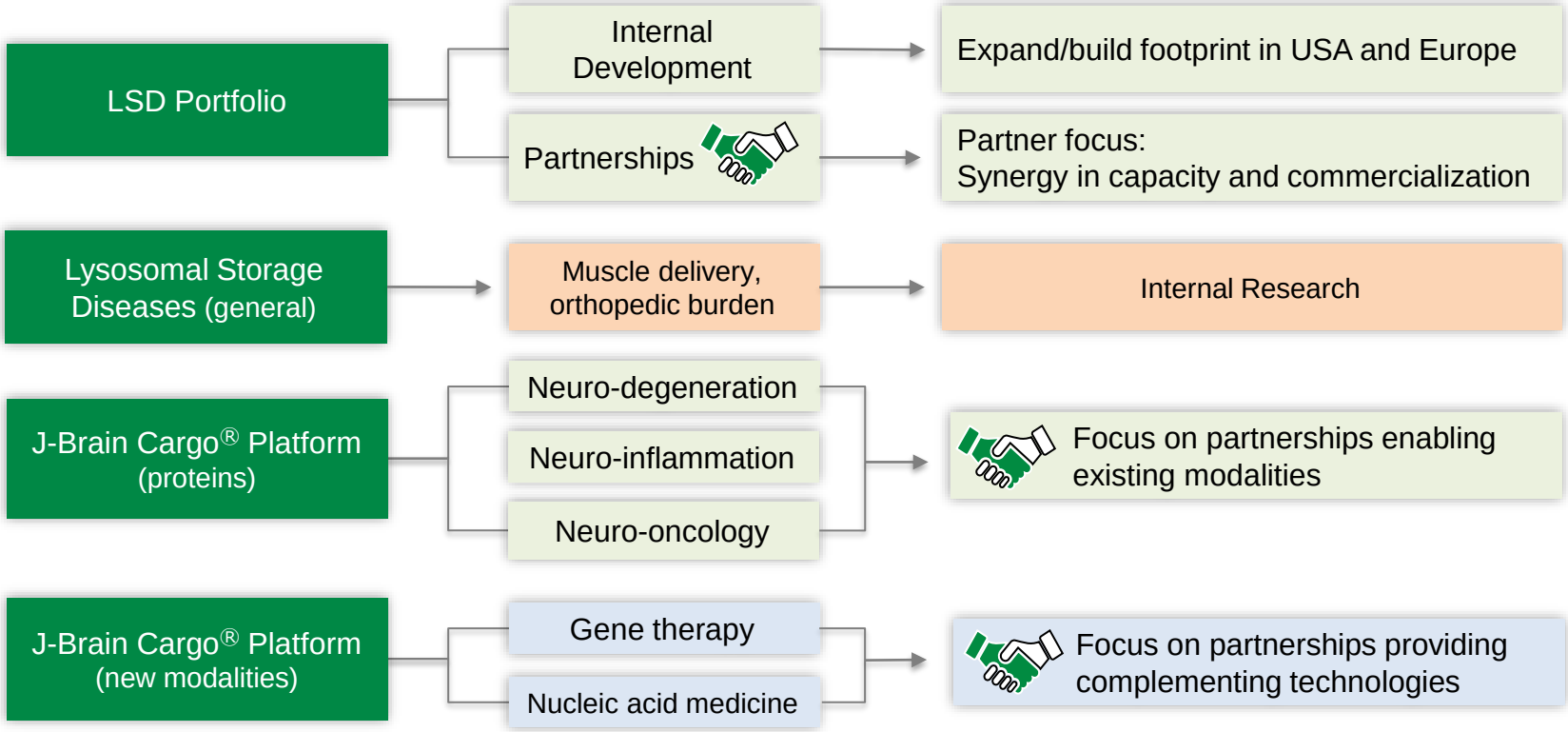
Currency: JPY

	FY2019 Results	First Year FY2020 Results	**Second year FY2021 Results**	Final year Forecast for FY2022	Guidance (Final year Goals)
Sales	24billion	**30 billion**	**51 billion**	**45.0 billion**	**32~36 billion**
Operating income	3.2billion	**8.2 billion**	**19.9 billion**	**14.5 billion**	**7~10 billion**
R&D expenditures	24.2%	**17.82%**	**14.0%**	**20.0%**	**Around 20 %**
Dividend Ratio	36.8%	**21.5%**	**18.8%**	**23.6%**	**Around 30 %***

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

Vision for Achieving the ”REVOLUTION”

Partnerships are at the core of JCR’s growth and acceleration strategy



Vision for Achieving the "REVOLUTION"

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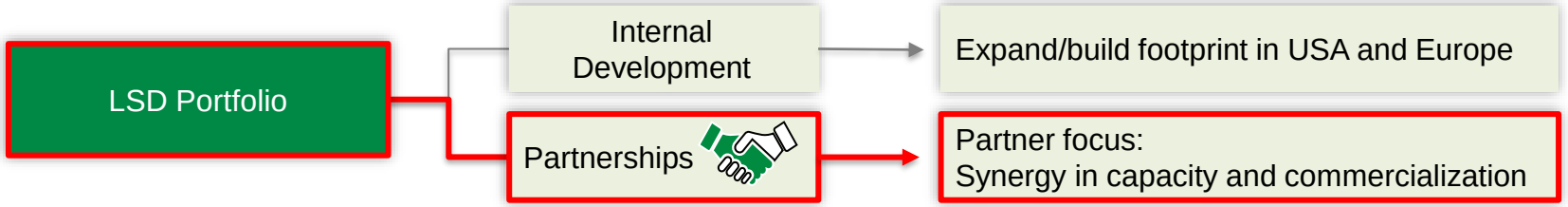


Indication	Status	Number of patients*1		Est. Market size*2	
		Japan	Worldwide	Japan(2019)	Worldwide(2019)
MPS II (Hunter syndrome) Takeda	Ph III	Approx. 250	Approx. 7,800	Approx. 7.6 billion JPY	Approx. 87.0 billion JPY
MPS I (Hurler syndrome etc.)	Ph I/II	Approx. 60	Approx. 3,600	TBD	Approx. 70.0 billion JPY
MPS IIIA (Sanfilippo type A)	FY2023～ Ph I	Approx. 30 (AB total)	Approx. 4,000	TBD	>70.0 billion JPY
MPS IIIB (Sanfilippo type B)	FY2023～ Ph I		Approx. 1,800		
MPS VII (Sly syndrome)	TBD	Several	Approx. 200	TBD	Approx. 9.8 billion JPY
GM2 gangliosidosis	～FY2025 Ph I	Approx. 30	TBD	TBD	TBD
Pompe diseaese	TBD	Approx. 80	Approx. 10,000	Approx. 3.0 billion JPY	Approx. 111.0 billion JPY

Source: JCR analysis *1 Number of patients: Calculated by JCR based on information published in the Ministry of Health, Labour and Welfare's research and others
*2 Market size: Internal analysis

Vision for Achieving the ”REVOLUTION”

Partnerships through deployment of J-Brain Cargo® are expected to become a major business pillar

Multi-Modality Innovator

contributing to a wide range of disease areas

J-Brain Cargo® Platform (proteins)

- Neuro-degeneration
- Neuro-inflammation
- Neuro-oncology

→ Focus on partnerships enabling existing modalities

J-Brain Cargo® Platform (new modalities)

- Gene therapy
- Nucleic acid medicine

→ Focus on partnerships providing complementing technologies

JCR's Sustainability : Efforts during "REVOLUTION"

Target **Realization of sustainability through activities based on RD･E･S･G**

Rare Disease

- Meeting the Challenge of Unmet Medical Needs
- Accelerating R&D
- Efforts to Raise Awareness

Environment

- Information Disclosure in Accordance with TCFD
- Environmental protection initiatives in factories under construction

Society

- Work environment that fosters compatibility of family with work
- Enhanced training programs for human resource development

Corporate Governance

- Establish a governance structure as a prime market company
- Improving the effectiveness of the Board of Directors

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"

FTSE Blossom Japan Sector Relative Index

JCR selected as a constituent of FTSE Blossom Japan Sector Relative Index

The FTSE Blossom Japan Sector Relative Index is created by global index provider FTSE Russell. It reflects the performance of Japanese companies that demonstrate strong environmental, social and governance (ESG) practices relative to their respective sectors and is designed to be sector neutral. To promote the transition to a low-carbon economy, companies with particularly high greenhouse gas emissions are included only if their improvement efforts are positively evaluated using the TPI Management Quality Score.

New challenges in the final year and beyond

- Maximize product value by maximizing global development potential.
- Establish alliances with partners capable of maximizing the value of J-Brain Cargo® technology as a new business pillar.
- Achieve sustainable growth through forward-looking investments.
- Develop a corporate culture of "Team JCR," shift to an appropriate personnel size and organizational structure that meets the medium- to long-term corporate image, and work to develop and secure human resources.

– REVOLUTION into the Future –

Appendix

Lysosomal diseases : Expected Timeline

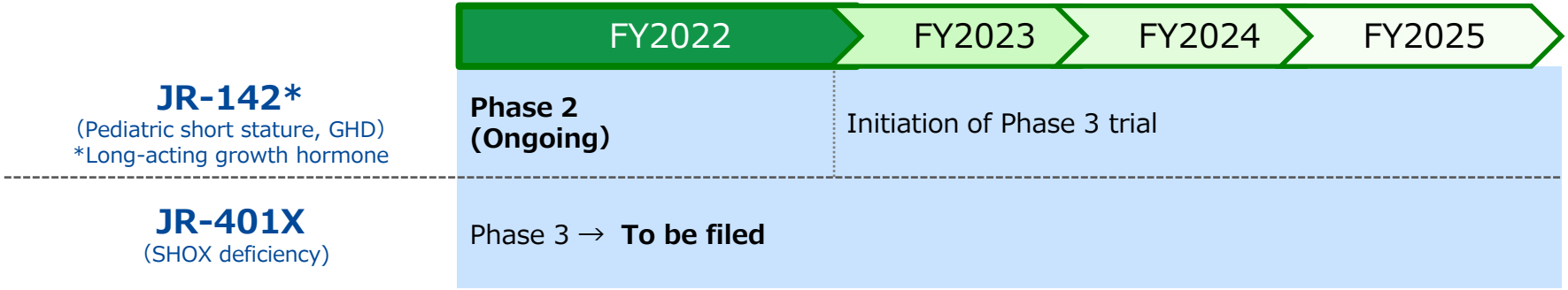
Note: It is a plan at this stage and is subject to change

	FY2022	FY2023	FY2024	FY2025	
JR-141 **pabinafusp alfa** (MPS II)	Brazil : Filed **Global : Phase 3 trial (Ongoing)**				• SAKIGAKE（PMDA） • Orphan Drug（PMDA） • Orphan Drug（FDA） • Fast Track（FDA） • Orphan Drug（EC） • PRIME（EMA）
JR-171 **lepunafusp alfa** (MPS I)	**Global : Phase 1/2 trial (Ongoing)**	Initiation of Phase 3 trial			• Orphan Drug (FDA) • Fast Track（FDA） • Orphan Drug（EC）
JR-441 (MPS IIIA)		Initiation of Phase 1/2 trial			• Orphan Drug（EC）
JR-162 (Pompe)	**Non-clinical (Ongoing)**				
JR-443 (MPS VII)	**Non-clinical (Ongoing)**				
JR-446 (MPS IIIB)	**Non-clinical (Ongoing)**	Initiation of Phase 1/2 trial			
JR-479 (GM2 gangliosidosis)	**Non-clinical (Ongoing)**		Initiation of Phase 1/2 trial		

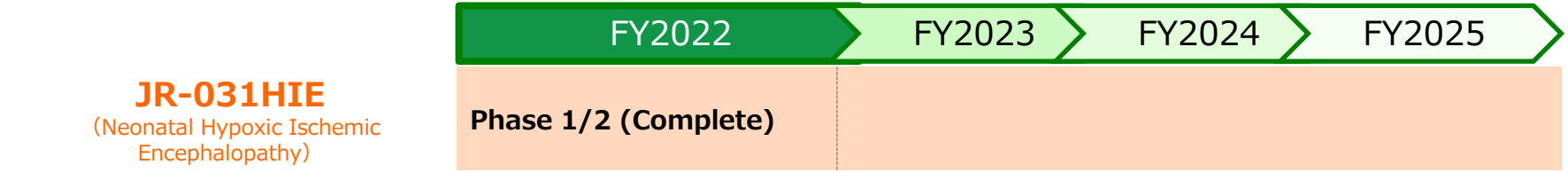
Other pipeline (Growth Hormone and regenerative medicine area)

Note: It is a plan at this stage and is subject to change

Expected timeline (Growth Hormone area)



Expected timeline (regenerative medicine area)



開発パイプライン（ライソゾーム病）

JR-141　pabinafusp alfa: BBB-penetrating iduronate-2-sulfatase (rDNA origin)

Indication：	MPS type II (Hunter syndrome)
Patient population*1：	250 (Japan) , 7,800 (WW) est.
Est. Market size*2：	7.6 billion JPY (2019 Japan), 87.0 billion JPY (2019 WW)
Disease overview：	Hunter syndrome is an X-linked recessive LSD caused by a deficiency of iduronate-2-sulfatase, an enzyme that breaks down glycosaminoglycans (mucopolysaccharides) in the body. MPS II gives rise to a wide range of somatic symptoms and central nervous system (CNS) symptoms.

JR-171　lepunafusp alfa: BBB-penetrating α-L-iduronidase (rDNA origin)

Indication：	MPS type I (Hurler, Hurler-Scheie, Scheie syndrome)
Patient population*1：	60 (Japan), 3,600 (WW) est.
Est. Market size*2：	28.0 billion JPY (2019 WW)
Disease overview：	MPS I is an autosomal recessive LSD caused by a deficiency of α-L-iduronidase, an enzyme that breaks down glycosaminoglycans (mucopolysaccharides) in the body. MPS I gives rise to a wide range of somatic and neurological symptoms. A major limitation to current ERT is that it does not address central nervous system (CNS) symptoms because of the enzyme’s inability cross the BBB.

*1 Calculated internally based on the date from MHLW and own research *2 Internal analysis

Progress of Other Compounds

JR-441 BBB-penetrating heparan N-sulfatase (rDNA origin)

Indication :	MPS type III A (Sanfilippo A syndrome)
Patient population*1 :	30 (Japan : Total of Type A&B) , 4,000 (WW) est.
Est. Market size*2 :	>70.0 billion JPY (2019 WW: Total of Type A&B)
Disease overview :	An autosomal recessive disease caused by a deficiency of the enzyme heparan-N-sulfatase that metabolizes mucopolysaccharides within the body. Notably, rapid progression of CNS disorders affects neurocognitive development, with a peak at 2 or 3 years of age. Type III A is relatively severe. Hematopoietic stem cell transplantation can be a treatment option, but its effectiveness remains to be established.

JR-162 J-Brain Cargo®-applied acid α-glucosidase (rDNA origin)

Indication :	Pompe disease
Patient population*1 :	80 (Japan), 10,000 (WW) est.
Est. Market size*2 :	3 billion JPY (2019 Japan), 110 billion JPY (2019 WW)
Disease overview :	An autosomal recessive disease caused by a deficiency of the enzyme acid α-glucosidase that causes an accumulation of Glycogen in muscle cells and nerve cells. The infantile onset manifests as suckling and muscle force lowering in postnatal 2 months. Natural history suggests a life expectancy of less than 18 months due to cardiac dysfunction and respiratory failure. Delayed onset cases present muscle weakness that involves respiratory muscles. Symptoms are multiple and systemic, including CNS disorders.

*1 Calculated internally based on the date from MHLW and own research *2 Internal analysis

Progress of Other Compounds

JR-443 BBB-penetrating β-glucuronidase (rDNA origin)

Indication :	MPS type VII（Sly syndrome）
Patient population*1 :	several (Japan) , 200 (WW) est.
Est. Market size*2 :	9.8 billion JPY est.（2019 WW）
Disease overview :	An autosomal recessive disease caused by deficiency of an enzyme, β-glucuronidase, that metabolizes mucopolysaccharides within the body, leading to accumulations of heparan sulfate and dermatan sulfate. Symptoms include bone deformation, joint contraction, as well as CNS disorders in severe cases. Hematopoietic stem cell transplantation and enzyme replacement therapy are treatment options, but their effectiveness, including that for CNS disorders remains to be established.

JR-446 BBB-penetrating α-N-acetylglucosaminidase (rDNA origin)

Indication :	MPS type III B（Sanfillipo B syndrome）
Patient population*1 :	30 (Japan : Total of Type A&B) , 1,800 (WW) est.
Est. Market size*2 :	>70.0 billion JPY（2019 WW: Total of Type A&B）
Disease overview :	An autosomal recessive disease caused by a deficiency of the enzyme α-N-acetylglucosaminidase that metabolize mucopolysaccharides within the body. Symptoms include accumulation of heparan sulfate in tissues throughout the body. Notably, it leads to rapid progression of CNS disorders, whereby neurocognitive development, with its peak around 2 or 3 years of age, deteriorates thereafter. Hematopoietic stem cell transplantation can be a treatment option, but its effectiveness remains to be established.

*1 Calculated internally based on the date from MHLW and own research *2 Internal analysis

Progress of Other Compounds

JR-142 Long-acting growth hormone (rDNA origin)

Indication :	Pediatric growth hormone deficiency
Note :	JCR's proprietary half-life extension technology, based on a novel modified albumin, allows significant increase in the half-life of various biotherapeutics (Patent filed)

JR-401X Somatropin (rDNA origin) (Expanded Indication of GROWJECT®)

Indication :	Short stature homeobox-containing gene (SHOX) deficiency
Prevalence* (Japan) :	450-500 est. per year

JR-031HIE Human mesenchymal stem cells (Expanded indication of TEMCELL®HS Inj.)

Indication :	Neonatal Hypoxic Ischemic Encephalopathy
Prevalence* (WW) :	2.5 of 1,000 live births (Target: 150-200 patients per year with moderate-severe disease indicated for therapeutic hypothermia as standard of care)

*Internal analysis

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.

FORWARD-LOOKING STATEMENT

The clinical development data mentioned in this document do not guarantee future results, nor do they guarantee the efficacy or effects of products under development.
This document is not intended to guarantee or advertise the efficacy of the product under development.
The clinical development data mentioned in this document include data not yet published in peer-reviewed academic journals or not yet presented at academic conferences. We will make them public in the future.
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The progress of clinical development may be affected by the pandemic of novel coronavirus infection (COVID-19) in the future .