FY2023 First-Half Results Briefing Session - Business Highlights -

October 26, 2022

JCR Pharmaceuticals Co., Ltd

【Securities code】4552, PRIME TSE

Agenda

- Domestic Sales Highlights
- Sustainability Highlights
- R&D Update

Highlights (May 2022- Oct. 2022)

2022

◆**May** - Public lecture to promote awareness of Mucopolysaccharidosis held

◆**Jul.** - Three committees for sustainability initiatives established

- Underwriting of a third-party allotment of new shares from Mycenax Biotech Inc., a CDMO in Taiwan, executed

- **JR-401X:** Application for expanded indication of GROWJECT® for short stature due to SHOX filed deficiency

◆**Aug.** - **JR-141:** Application for manufacturing and marketing approval in Brazil denied

- Upgrading of Melon Nikki™ , a medication management app for pediatric patients receiving GROWJECT® treatment

◆**Sep.** - Disposition of treasury stock through third-party allotment made for donation to Kyoto University

- JCR Europe B.V. as overseas subsidiary established to engage in development activities in Europe

- METI “Project for Establishing Biopharmaceutical Manufacturing Sites to Strengthen Vaccine Production” selected

- AlliedCel Corporation, a joint venture with Sysmex specialized in regenerative medicines, established

◆**Oct.** - "Kurumin" certification in recognition of a company that supports childcare granted to JCR for two consecutive terms

- Decision to construct a new formulation plant

Update on domestic sales of main products

GROWJECT®

- Sales declined through 2Q due to NHI price revision (-8% approx.).
- Sales volume remained steady.
- Jul. Filed for GROWJECT® for short stature due to SHOX-deficiency.
- Aug. Upgraded Medication Management App Melon Nikki™. JCR continues to implement measures based on its comprehensive device strategy.

IZCARGO®

- 10 new patients have started treatment with IZCARGO® so far.
- Most new cases are younger than 6 years of age.
- Steady Information delivery and collection activities leading to the administration of IZCARGO® are underway, such as discussing the administration policy for attenuated patients with medical professionals.

TEMCELL®

- As many cases administered were pediatric, the number of bags used per patient was relatively low.
- A decrease in new donor registrations in bone marrow banks has been reported due to COVID-19.

JCR's Sustainability

Jul. 2022: Three committees for sustainability initiatives established

- **Sustainability Advisory Committee**
- **Sustainability Committee (In charge: Toru Ashida)**
- **Environmental Committee (In charge: Yoshio Hiyama)**

Currently the Sustainability Committee is taking the lead in identifying our material issues.

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

Major initiatives in FY2022 1st Half

Rare Disease	Environment	Society	Corporate Governance
• Public lectures to raise awareness both internally and externally.	• Completed Information Disclosure in Accordance with TCFD Recommendations • Promoted environmental conservation initiatives at a new plant under construction	• Launch of "JCR Academy," a global human resources development training program • Obtained Kurumin for two consecutive terms	• Addition of two outside directors as part of the governance structure

Agenda

- Domestic Sales Highlights
- Sustainability Highlights
- R&D Update

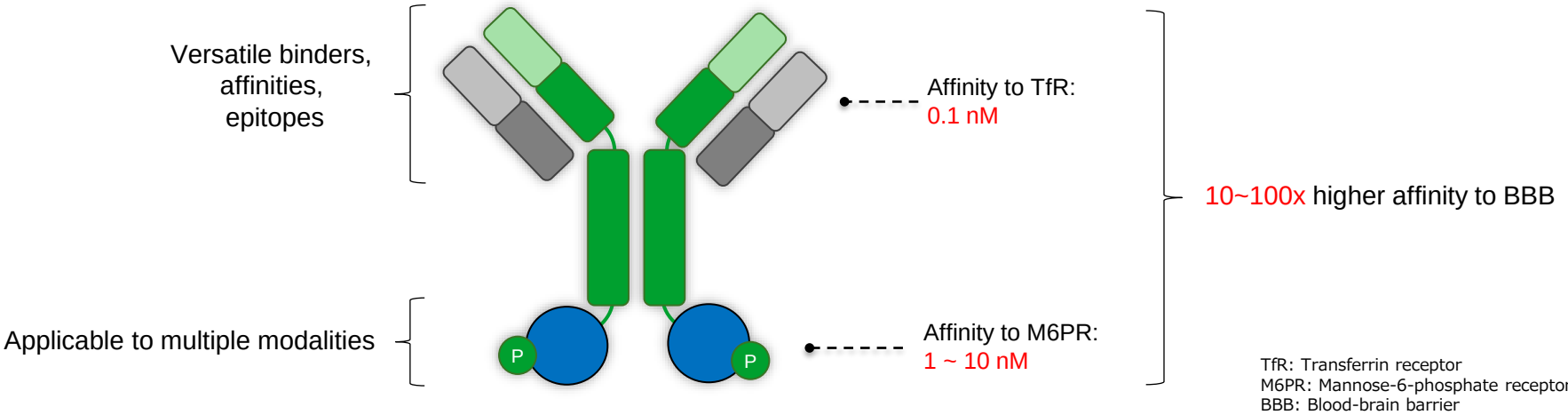
Development Portfolio Overview

★ : progress in development stage since May 2022

Code	Indication	Preclinical	Clinical Trials	Filed	Approved	Remarks/ Time to next value inflection point
JR-141	MPS type II (Hunter Syndrome)		**Approved**			• J-Brain Cargo® • FY2025～FY2027 Approval in US, EU, Brazil
			Phase 3			
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		**Filed**			• Expanded indication of GROWJECT® • FY2023 approval in Japan
★ **JR-142**	Pediatric growth hormone deficiency		**Phase 2** (Completed patient recruitment)			• 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

Design Uniqueness of the J-Brain Cargo® Technology



TfR: Transferrin receptor
M6PR: Mannose-6-phosphate receptor
BBB: Blood-brain barrier

Differentiator	Why does it matter?
Preferential BBB targeting	• Higher uptake to brain compared to somatic tissue
High affinity	• Lower doses resulting in shorter infusion times, manageable infusion reactions
Versatility in binders, affinities, epitopes, modalities	• Customizable to different diseases and modalities
Safety	• Best characterized safety profile in the industry

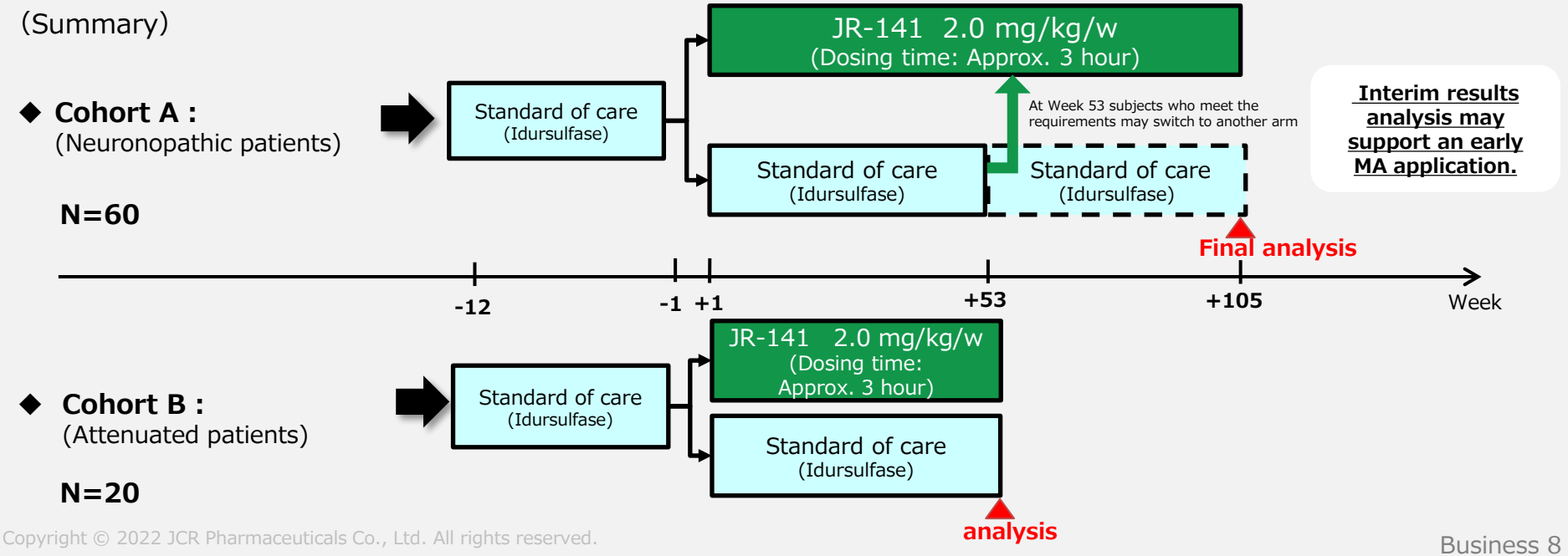
Progress on JR-141 (JR-141-GS31)

JR-141

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

- **As of Oct. 2022:**
  Expansion of clinical trial sites in the US, Brazil, and Europe and patient enrollment is in progress. Also, prepare the initiation of the study in several new areas.

(Summary)



Interim results analysis may support an early MA application.

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

JR-141

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

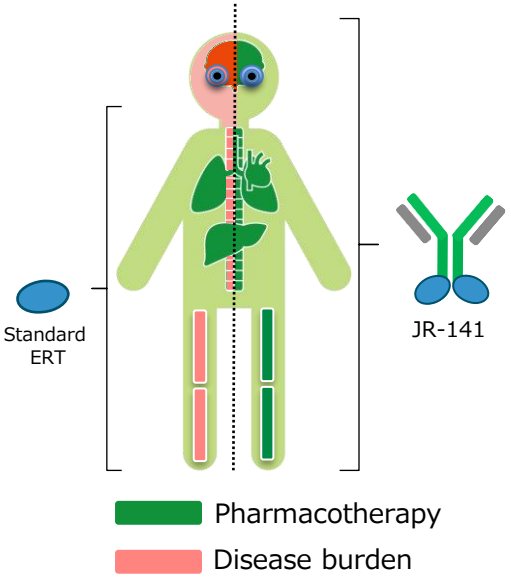
Objective : JR-141-GS31

1. **To demonstrate the significant efficacy of JR-141 on CNS signs and symptoms in MPS-II subjects relative to standard ERT.**

   JR-141 is expected to have superior activity on neurologic signs and symptoms of MPS-II by reducing substrate in the brain.

2. **To demonstrate control of somatic signs and symptoms by JR-141 that is comparable to standard ERT.**

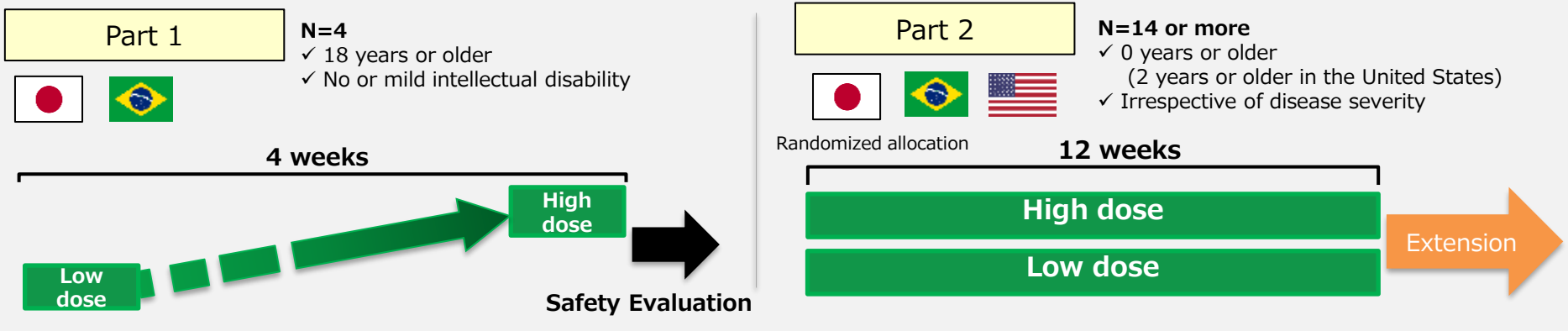
   JR-141 is expected to control somatic symptoms and biomarkers comparable to standard ERT (even though some improved symptom control may be seen due to dual uptake mechanism by JR-141)



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)

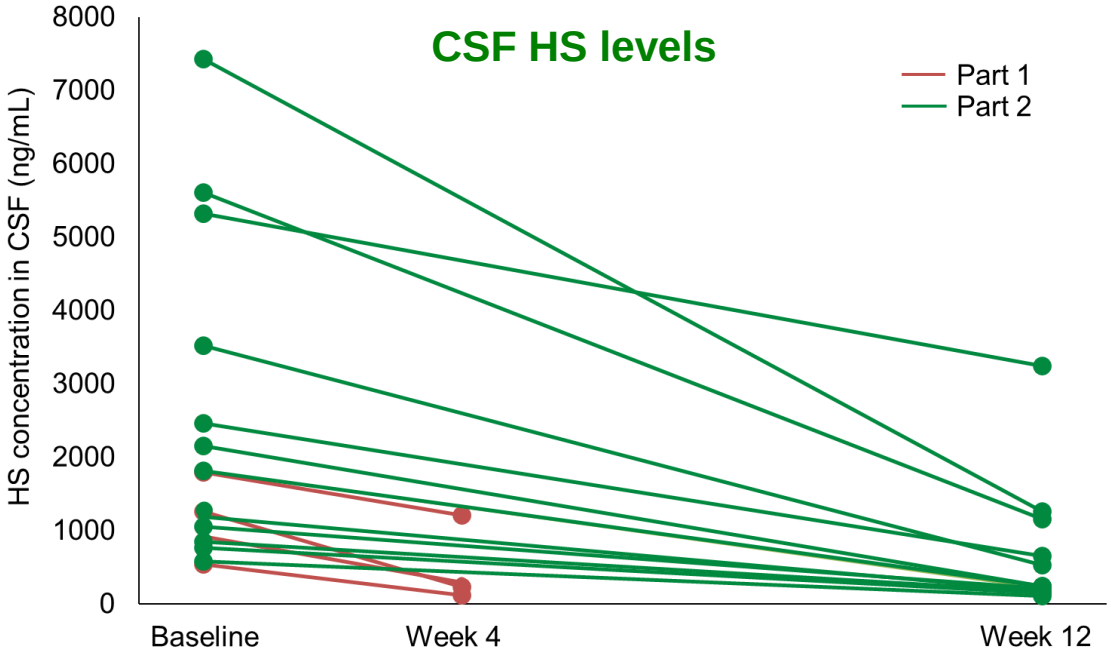


	Part 1	Part 2
Primary endpoint	Safety	
Secondary and exploratory endpoints	・Plasma drug concentration, pharmacokinetic parameters ・Exploratory efficacy for central nervous system and systemic symptoms	
Geography	Japan・Brazil	Japan・Brazil・USA
Clinical trials identifier	clinicaltrials.gov NCT04227600	

Progress on JR-171

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

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



A biomarker response was observed in 100% of patients

CSF : Cerebrospinal fluid
HS : Heparan sulfate

JCR has built International Clinical Development Capacities

Sep. 2022 : Establishment of JCR Europe as Overseas Subsidiary to Become a Base for Development in Europe

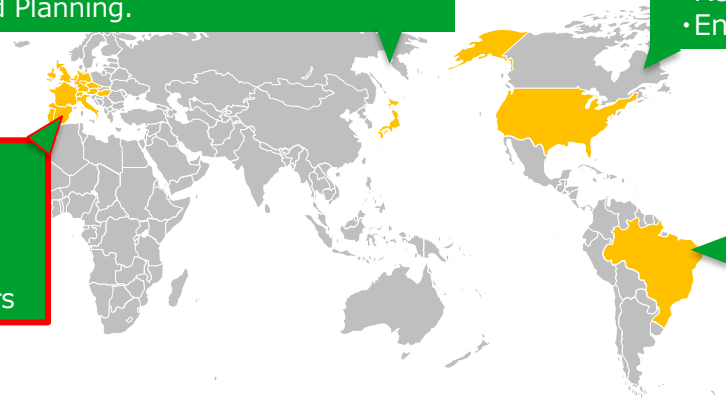
To strengthen global clinical development capacity, know-how and physical presence in Europe.

JR-141 (IZCARGO®) : SAKIGAKE
JR-141 (IZCARGO®) : Orphan drug

Japan : JCR pharmaceuticals
- Domestic and international clinical development
- Operations and Planning.

USA : JCR USA
- Clinical Operations, CRO management
- Regulatory Affairs
- Enhance the network with stake holders

JR-141 : Orphan drug
JR-141 : Fast track
JR-171 : Orphan drug
JR-171 : Fast track



Europe : JCR Europe
- Clinical Operations
- Regulatory Affairs
- Medical Affairs
- Enhance the network with stake holders

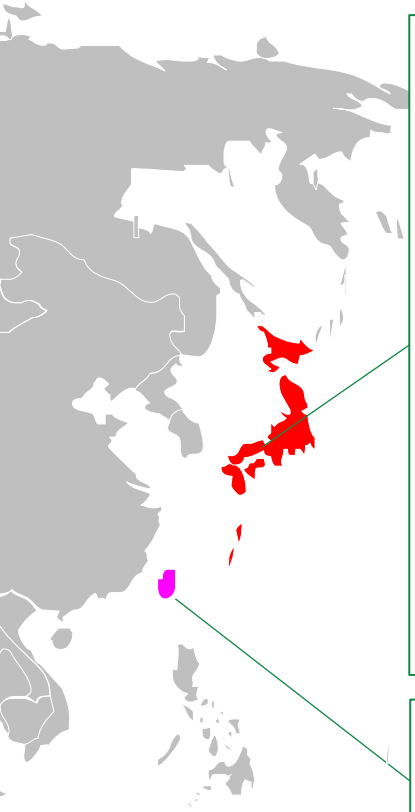
JR-141 : Orphan drug
JR-141 : PRIME
JR-171 : Orphan drug
JR-441 : Orphan drug

Brazil : JCR DO BRASIL
- Regulatory Affairs
- CRO management
- Medical Affairs
- Enhance the network with stake holders

JR-141 : RDC
JR-171 : RDC

Biomanufacturing capabilities

Expanding manufacturing capacity for global supply



Japan:

Established Process Development and Biomanufacturing capacities ensure process control and cost-efficient manufacturing of clinical and commercial supply.

Site	Single-use or Stainless Bioreactor Capacity
Murotani	2x 400L; 2x 80L
	2x 2,000L; 2x 200L
Kobe API	2x 2,000L; 2x 200L
Kobe	Filling, visual inspection, packaging
Seishin	Human mesenchymal stem cells
Kobe Science Park Center	4x 2,000L, 4x 200L (planned)
合計	**8x 2,000L; 2x 400L; 8x 200L, 2x 80L**

Taiwan:

Mycenax Biotech Inc. has affinity for JCR's products, and capital participation will expand the flexibility of JCR's production capacity.

Expansion of JCR's Manufacturing Facilities

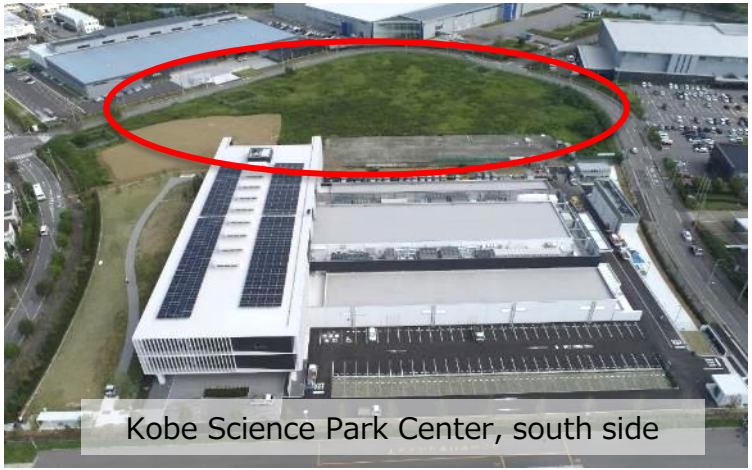
Kobe Science Park Center (under construction)

- Utilize subsidy related to the extraordinary grant for establishment of a production system for COVID-19 vaccines
- Completion of construction: November 2022 (Scheduled)
- Start of operation: FY2023 or later

Kobe Science Park Center, west side

New formulation plant (resolved on 10/26/2022)

- Adjacent to Kobe Science Park Center (land acquisition in March 2022)
- Utilize the Ministry of Economy, Trade and Industry's "Subsidy for Adoption of Vaccine and Other Production System Improvement Project" (subsidy amount to be determined)



Kobe Science Park Center, south side

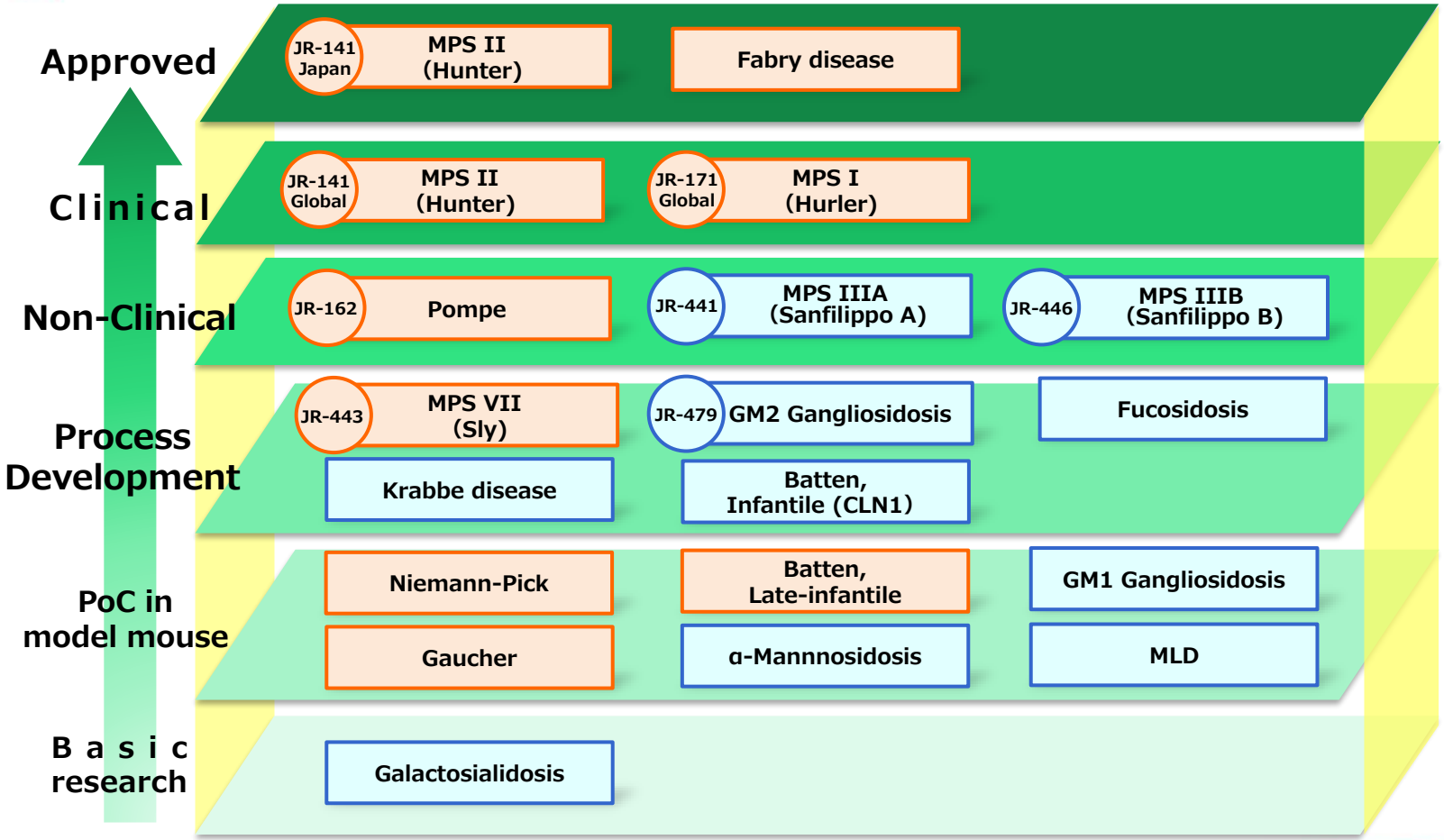
Both plants will manufacture JCR's products in the absence of requests to manufacture vaccines etc.

Lysosomal diseases : Expected Timelines

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

	FY2022	FY2023	FY2024	FY2025	
JR-141 **pabinafusp alfa** (MPS II)	**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 (Data analysis)**	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	

JCR's LSD Pipeline comprises 18 Programs

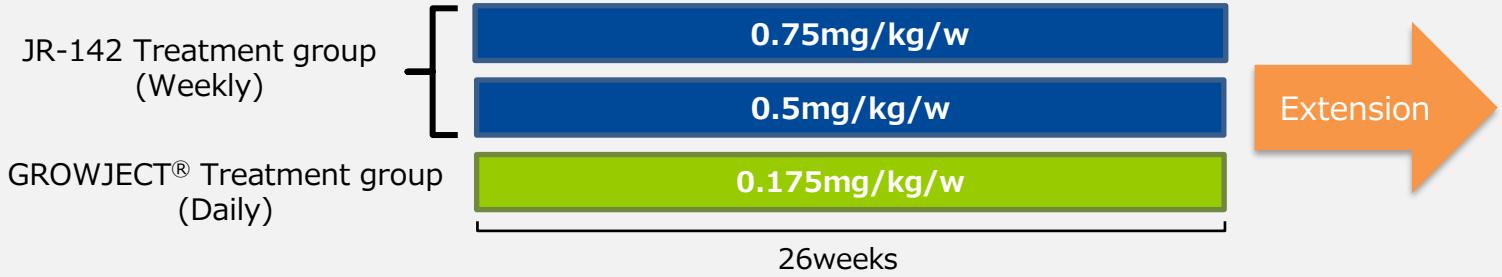


Progress on JR-142

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

Phase 2 clinical Trial (JR-142-201): Brief Summary

◆ **Completed patient recruitment**
(First Patient Dosed: May 2021)

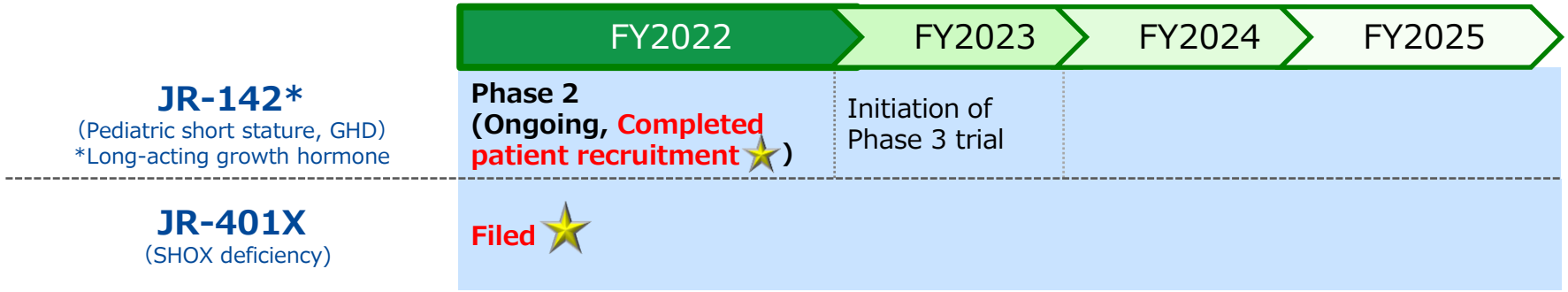


Subjects	Pediatric Growth Hormone Deficiency (Pediatric GHD)
Endpoints	• Assessment of PK/PD profile • Growth rate (after 26 weeks) • Safety etc.
Number of Subjects	24subjects
Study drug	JR-142 / GROWJECT® *Both are administered by auto-injector.
Details	jRCT(Identifier : jRCT2031200372)

Growth Hormone area: Expected Timelines

Note: It is a plan at this stage and is subject to change
★ : progress in development stage since May 2022

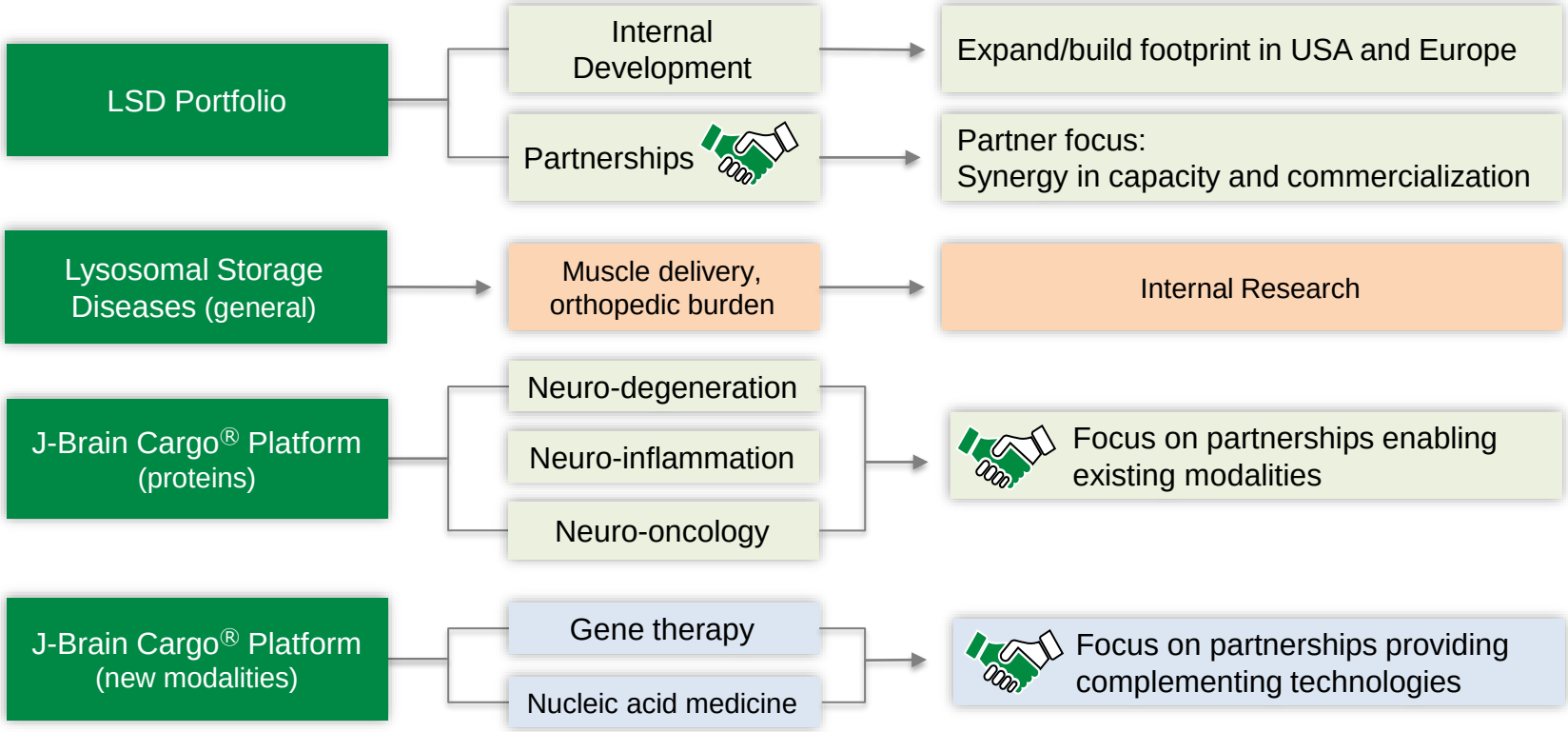
Expected timeline (Growth Hormone area)



Leveraging Growth Opportunities from J-Brain Cargo® Technology

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

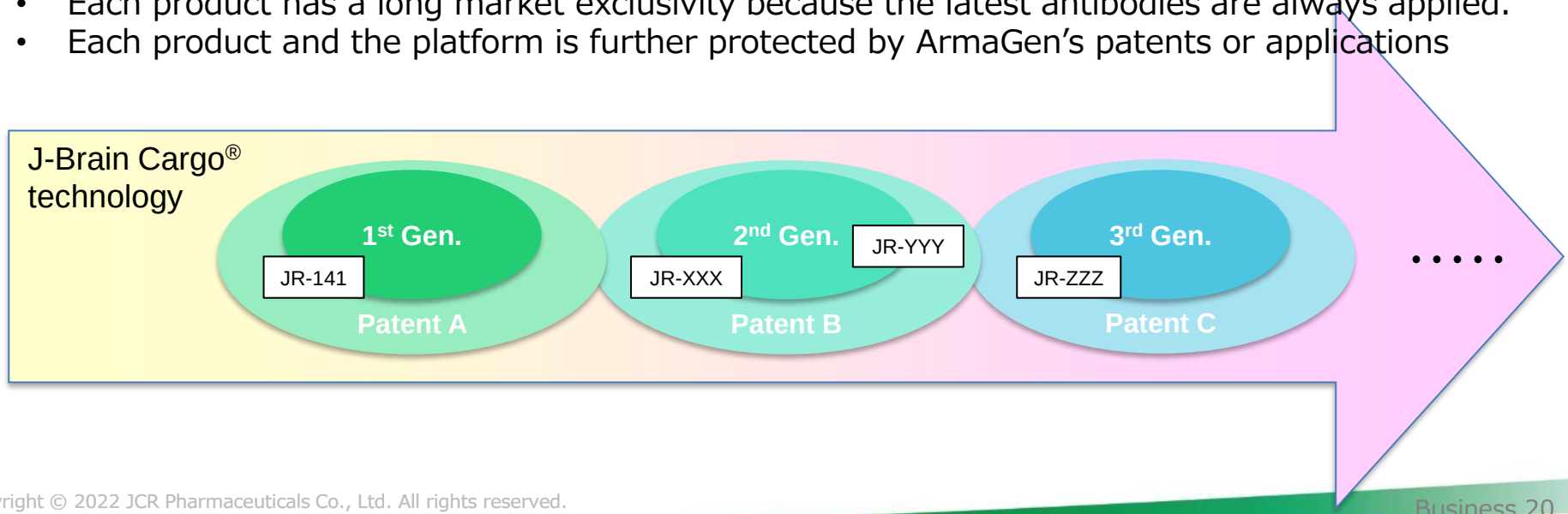
LSD: Lysosomal diseases
CNS: Central central nervous system



To be at the forefront of BBB targeting technology area

JCR continues to be at the forefront of BBB targeting technology area by constantly taking on the challenge of obtaining new antibodies and protecting them with appropriate IR strategy.

- New antibodies using the J-Brain Cargo® technology are constantly being obtained. And each of them is steadily protected by appropriate patents or applications.
- Each product has a long market exclusivity because the latest antibodies are always applied.
- Each product and the platform is further protected by ArmaGen's patents or applications



Establish a Joint Venture “AlliedCel Corporation”

Oct. 3, 2022: Sysmex Corporation and JCR have established AlliedCel Corporation for carrying out research and development, manufacture and sales of cell-based regenerative medicine products including hematopoietic stem cells and other stem cells.

AlliedCel

sysmex
Lighting the way with diagnostics

Expertise in quality control testing technology and knowledge of workflows efficiency using robotics technology, including IoT.

JCR Pharmaceuticals

Expertise in developing, manufacturing and marketing regenerative medicine products,

Location :	1-5-5 Minatojimaminami-machi, Chuo-ku, Kobe, Hyogo, Japan
Capital :	100 million JPY
Capital reserve :	100 million JPY
Investment ratio :	Sysmex 50%, JCR 50%
Executive officers :	President: Hiroyuki Sonoda (Vice President, Research and Corporate Strategy, Executive Director of Research Division, JCR Pharmaceuticals Co., Ltd.) Executive Vice President, Member of the Managing Board: Kenji Tsujimoto (Executive Vice President of Technology Strategy Division, Sysmex Corporation)
Business content :	Research and development, manufacture and sales of regenerative medicine products

– 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.

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.
In accordance with the Fair Disclosure Rules, data other than those listed in this document will not be disclosed in questions and answers. We appreciate your understanding.
The progress of clinical development may be affected by the pandemic of novel coronavirus infection (COVID-19) in the future .

Appendix

Pipeline of LSDs

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

Pipeline of LSDs

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

Pipeline of LSDs

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

Pipeline of LSDs

JR-479 BBB-penetrating β-Hexosaminidase A

Indication :	GM2 gangliosidosis (Tay-Sachs disease, Sandohoff disease)
Patient population*1 :	30 (Japan), TBD(WW) est.
Est. Market size*2 :	TBD
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.

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

Pipeline 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

Lysosomal diseases : Expected Timelines

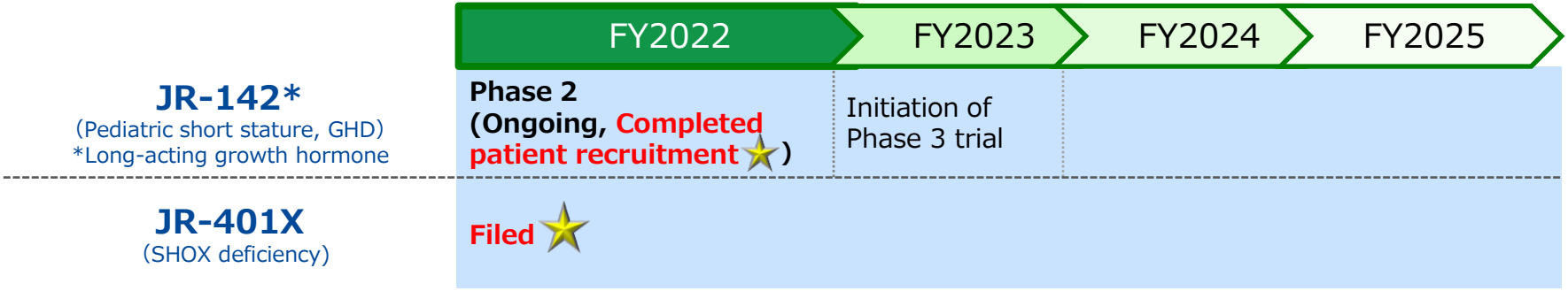
Note: It is a plan at this stage and is subject to change

	FY2022	FY2023	FY2024	FY2025	
JR-141 **pabinafusp alfa** (MPS II)	**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 (Data analysis)**	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
★: progress in development stage since May 2022

Expected timeline (Growth Hormone area)



Expected timeline (regenerative medicine area)