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FY2023 First-Half Results Briefing Session

November 2, 2023

JCR Pharmaceuticals Co., Ltd.

【Securities code】4552, PRIME. TSE
【Contacts】ir-info@jp.jcrpharm.com

Agenda

1. JCR’s Goal in Mid 2030s

2. FY2023 1st-Half Financial Results

3. JCR Activities for Further Growth
    1. Domestic sales products
    2. R&D

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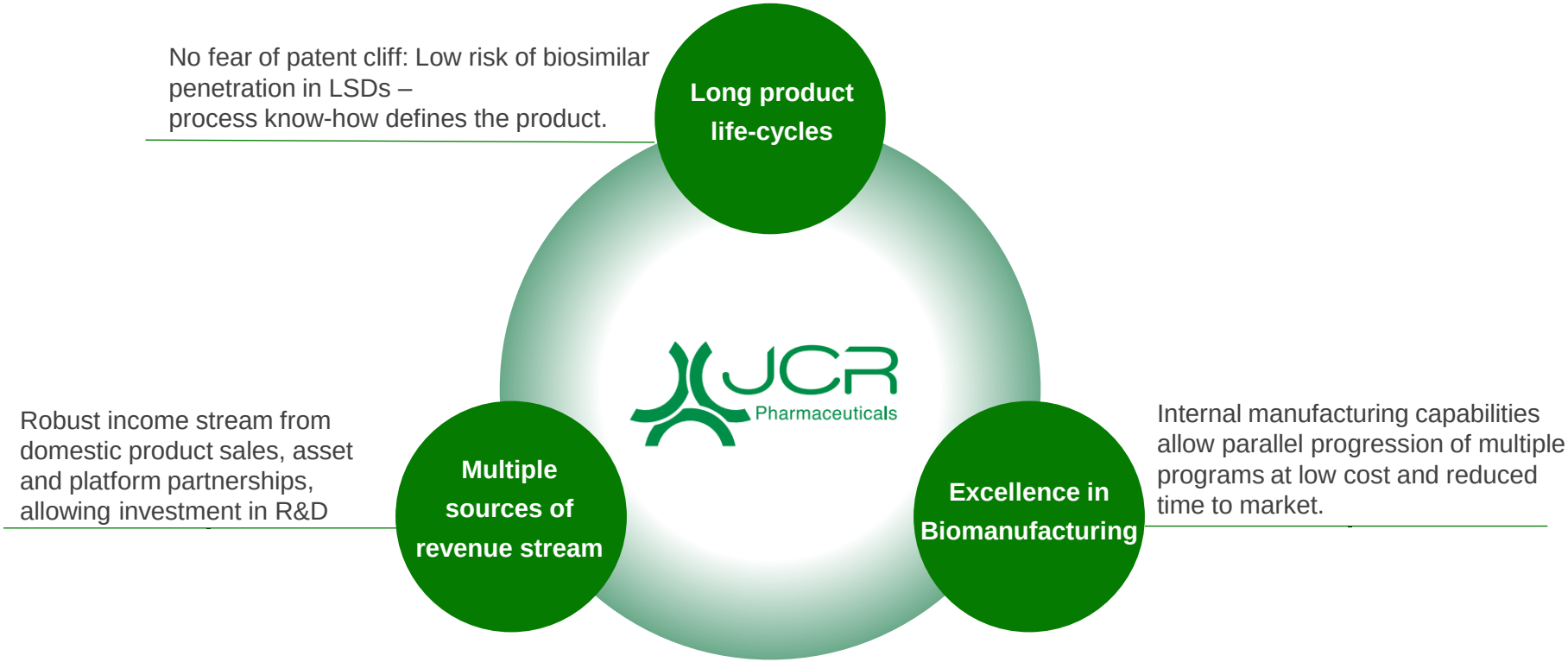
JCR's Goal in Mid 2030s

Yoh Ito
Senior Corporate Officer, Corporate Strategy

JCR will continue to focus on Rare Diseases



Very few pharmaceutical companies in the field of rare diseases are continuously profitable worldwide.

JCR has the business infrastructure to make this possible.

JCR's Revenue Growth Strategy

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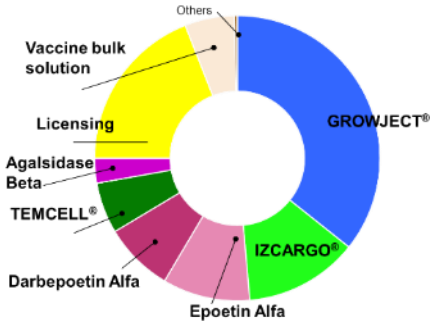
1 Domestic Products



KISSEI　Sumitomo Pharma

Continuous Revenue Stream
to sustain investment into growth areas

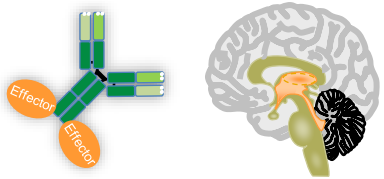
2 Portfolio Assets

Takeda　MEDIPAL

Advance existing portfolio
to leverage revenue potential in LSD space

Target: Rare diseases

3 Platform Technologies



Neuro-Degeneration, neuro-oncology, neuro-muscular, ocular diseases

Sumitomo Pharma　Takeda
ALEXION AstraZeneca Rare Disease　Angelini Pharma

Entry into indications with exponential revenue potential

Target: Severe diseases

More Stable Profit Structure

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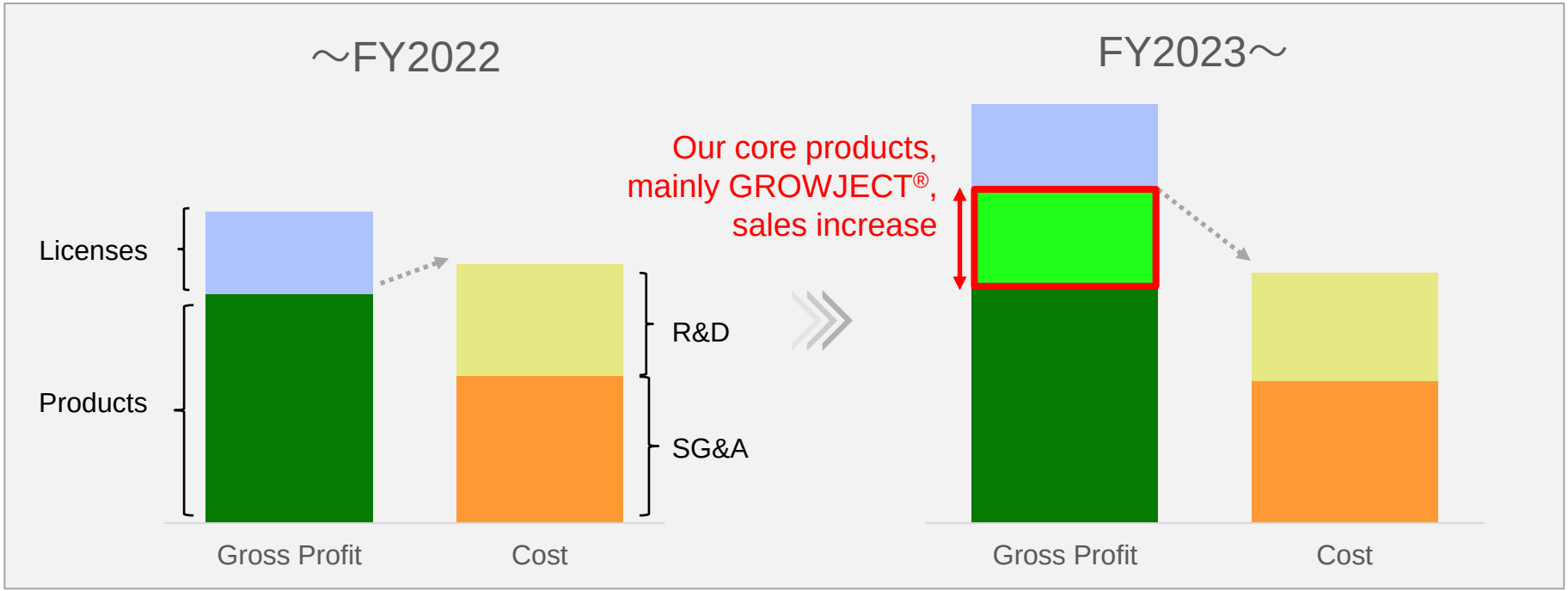
Enhanced sales from GROWJECT® enabled a more stable profit structure, allowing to cover R&D and SG&A expenses solely through revenue from domestic products.



Estimated Timeline to Approval and Revenue from LSD Assets

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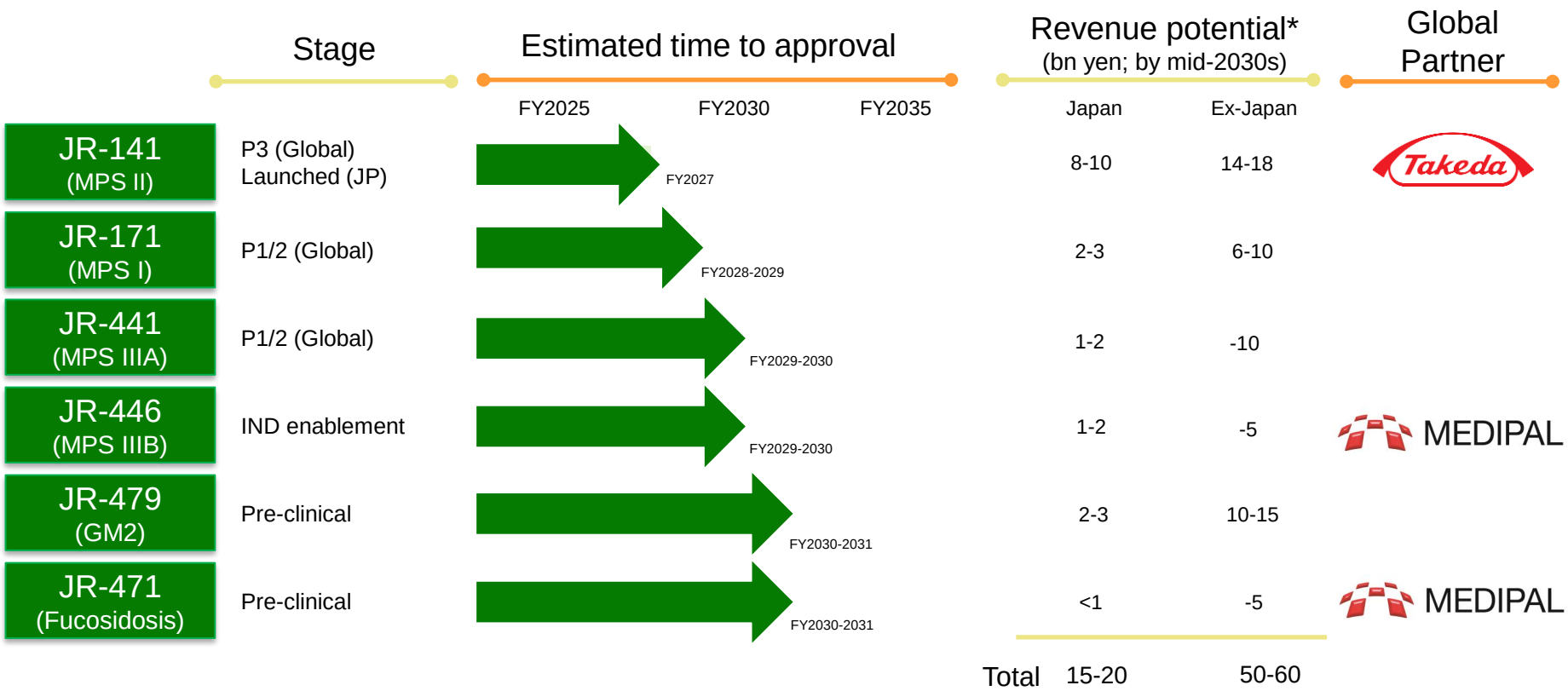

* Calculated by JCR based on est. market

J-Brain Cargo® as Door-Opener into broader CNS Indications

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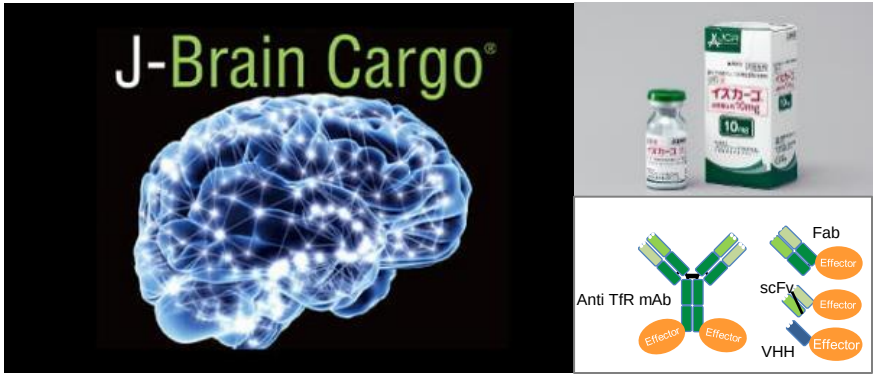
J-Brain Cargo® **to deliver drugs into the CNS**



50 million patients (WW)
$57 billion

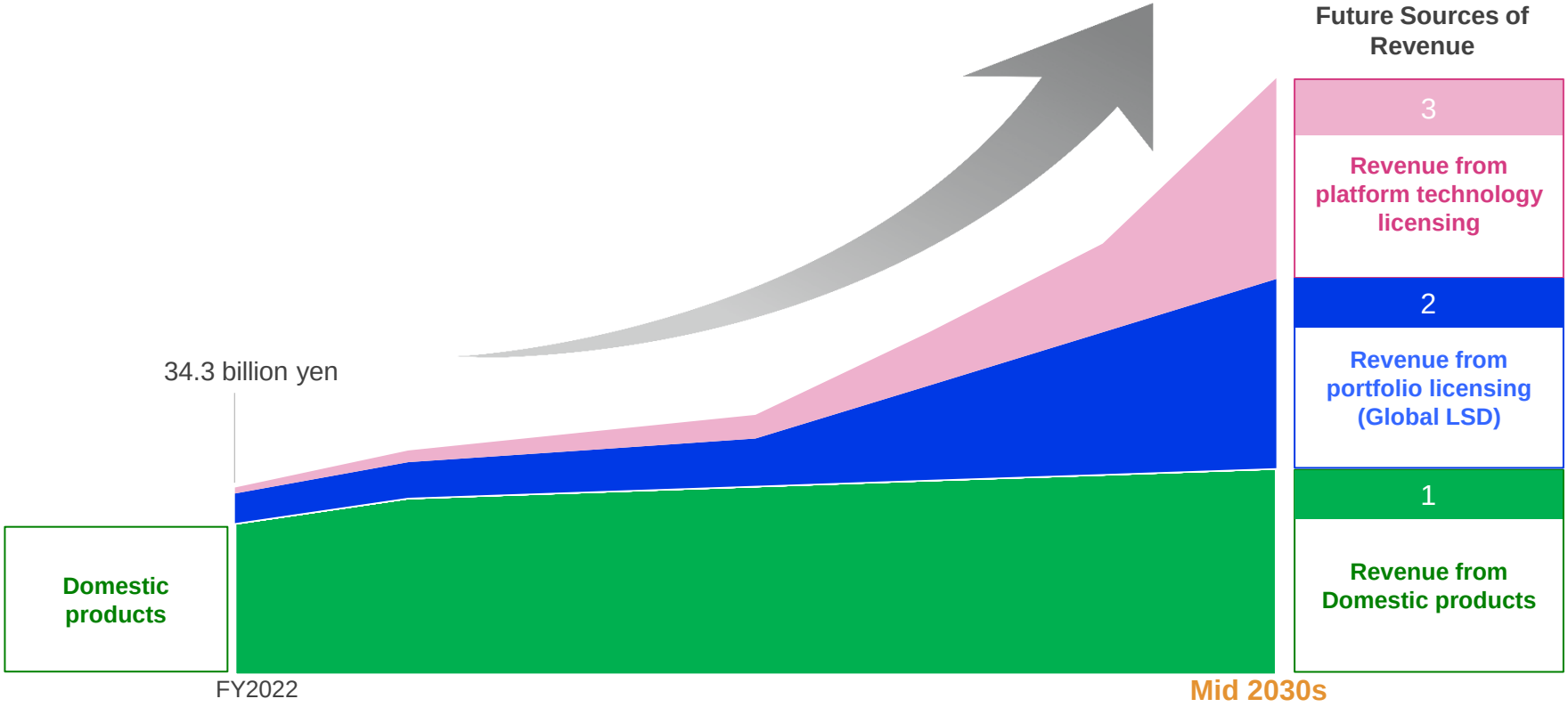
Neurodegenerative Diseases

- **Alzheimer's Disease**
- **Parkinson's Disease**
- **Epilepsy**
- ALS
- Multiple Sclerosis
- SMA
- Huntington Disease

30,000 patients (WW)
$10billion

LSD

Be a 100 bn Yen Company by mid-2030s based on Three Pillars



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FY2023 1st-Half Financial Results

Yoshihiro Oota
Director, Accounting Department, Corporate Strategy Division

Financial Highlights（Apr. 1, 2023- Sep. 30, 2023）

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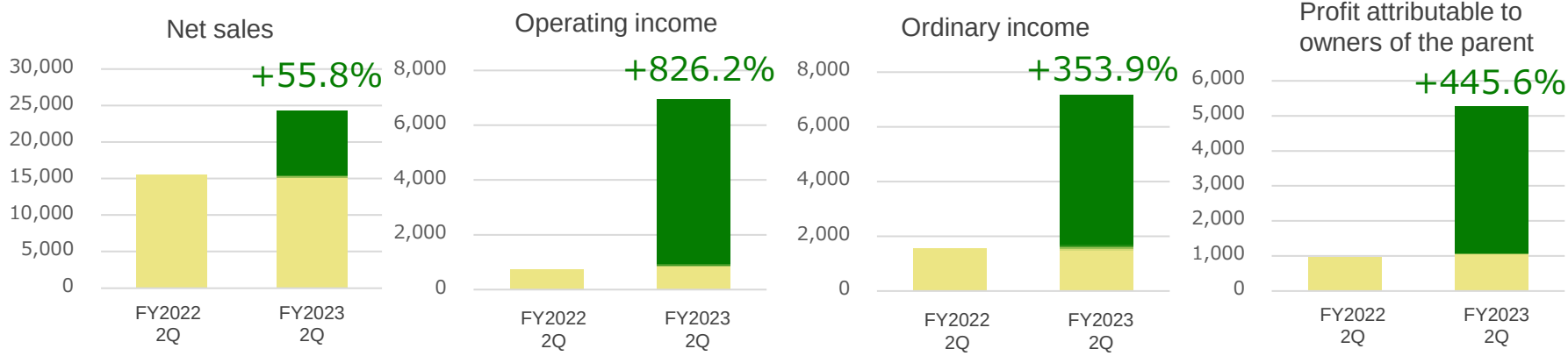
Strong sales of core products and higher contract income contributed to a significant year-on-year increase in revenues and profits.

- Net sales : 24,272million yen （YoY +55.8%）
- Operating income : 6,898million yen （YoY +826.2%）
- Ordinary income : 7,126million yen （YoY +353.9%）
- Profit attributable to owners of the parent : 5,253million yen （YoY +445.6%）

(Unit: million yen)



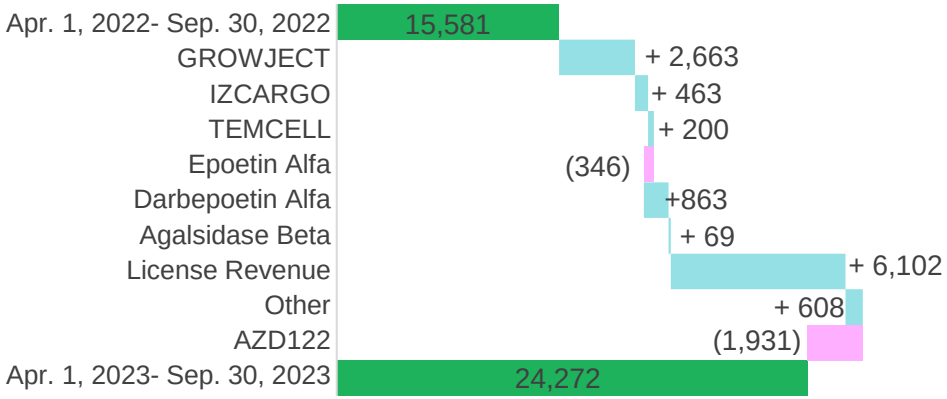
Breakdown of Net Sales

(Unit: million yen)

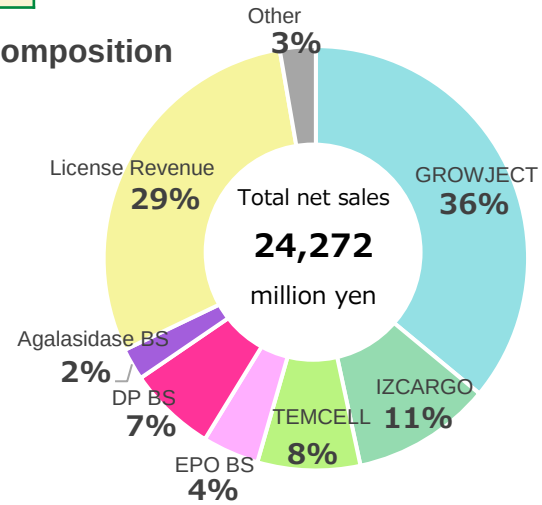
	FY2022	FY2023				
	Six months ended Sep. 30, 2022	Six months ended Sep. 30, 2023	YoY		Full year Forecast (Revised)	Progress Rate
			Difference	Ratio		
Core products	12,583	**16,495**	+3,913	+31.1%	34,700	**47.5%**
License Revenue	1,010	**7,112**	+6,102	+604.2%	8,100	**87.8%**
Other	56	**664**	+608	+1085.7%	2,600	**25.5%**
AZD1222 bulk	1,931	**—**	(1,931)	—	—	**—**
Total net sales	15,581	**24,272**	+8,691	+55.8%	45,400	**53.5%**

- Core products sales increased 31.1% YoY due to strong sales of GROWJECT® and other core products, IZCARGO® and TEMCELL®.
- License revenue and other sales (including contract manufacturing) progressed as planned, resulting in a YoY increase.

Total net sales （Unit：million yen）



Sales Composition



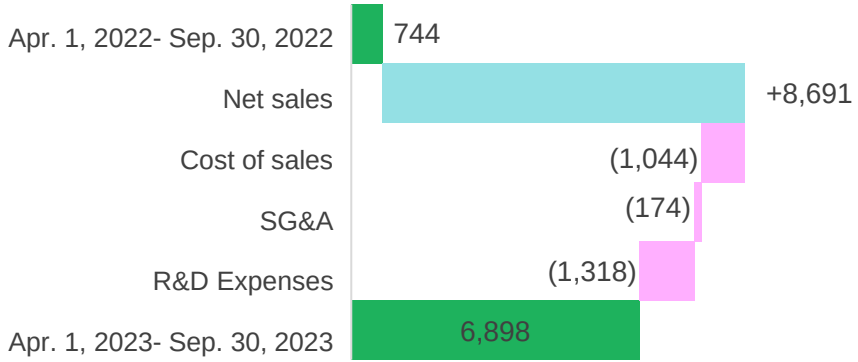
Operating Income

(Unit: million yen)

Consolidated	FY2022	FY2023				
	Six months ended Sep. 30, 2022	Six months ended Sep. 30, 2023	YoY		Full year Forecast (Revised)	Progress Rate
			Difference	Rate		
Net Sales	15,581	24,272	+8,691	+55.8%	45,400	53.5%
Cost of sales	4,836	5,881	+1,044	+21.6%	12,400	47.4%
Gross Profit	10,744	18,391	+7,646	+71.2%	33,000	55.7%
SG&A	5,782	5,957	+174	+3.0%	12,800	46.5%
R&D	4,216	5,535	+1,318	+31.3%	9,700	57.1%
Operating income	744	6,898	+6,153	+826.2%	10,500	65.7%

	Six months ended Sep. 30, 2022	Six months ended Sep. 30, 2023
Ratio of cost of sales	31.0%	24.2%
Ratio of cost of SG&A	37.1%	24.5%
Ratio of cost of R&D	27.1%	22.8%
Operating income ratio	4.8%	28.4%

Operating income （Unit：million yen）



- With the growth in net sales, operating income increased significantly by 826.2% YoY.
- As a result of active R&D activities, R&D expenses increased 31.3% to 5,535 million yen (up 1,318 million yen YoY).

JCR Activities for Further Growth
1. Domestic sales products

Toru Ashida
Senior Vice President, Sales

Sales of Core Products

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Strong sales of core products contributed significantly to the large increase in profit and sales.

	FY2022		FY2023				
	Full Year	Six months ended Sep. 30, 2022	Six months ended Sep. 30, 2023	YoY Difference	YoY Rate	Full year Forecast (Revised)	Progress Rate
GROWJECT®	12,261	6,083	**8,746**	+2,663	+43.8%	19,500	**44.9%**
IZCARGO®	4,428	2,118	**2,581**	+463	+21.9%	5,500	**46.9%**
TEMCELL®HS Inj.	3,404	1,701	**1,901**	+200	+11.8%	3,300	**57.6%**
Treatments for renal anemia	4,696	2,157	**2,674**	+517	+24.0%	5,000	**53.5%**
Epoetin Alfa BS Inj. [JCR]	2,710	1,392	**1,046**	(346)	(24.9%)	2,200	**47.5%**
Darbepoetin Alfa BS Inj. [JCR]	1,986	765	**1,628**	+863	+112.8%	2,800	**58.1%**
Agalsidase Beta BS I.V. Infusion [JCR]	964	521	**590**	+69	+13.2%	1,400	**42.1%**
Total Core products	25,755	12,583	**16,495**	+3,913	+31.1%	34,700	**47.5%**

GH Market Trends in Japan

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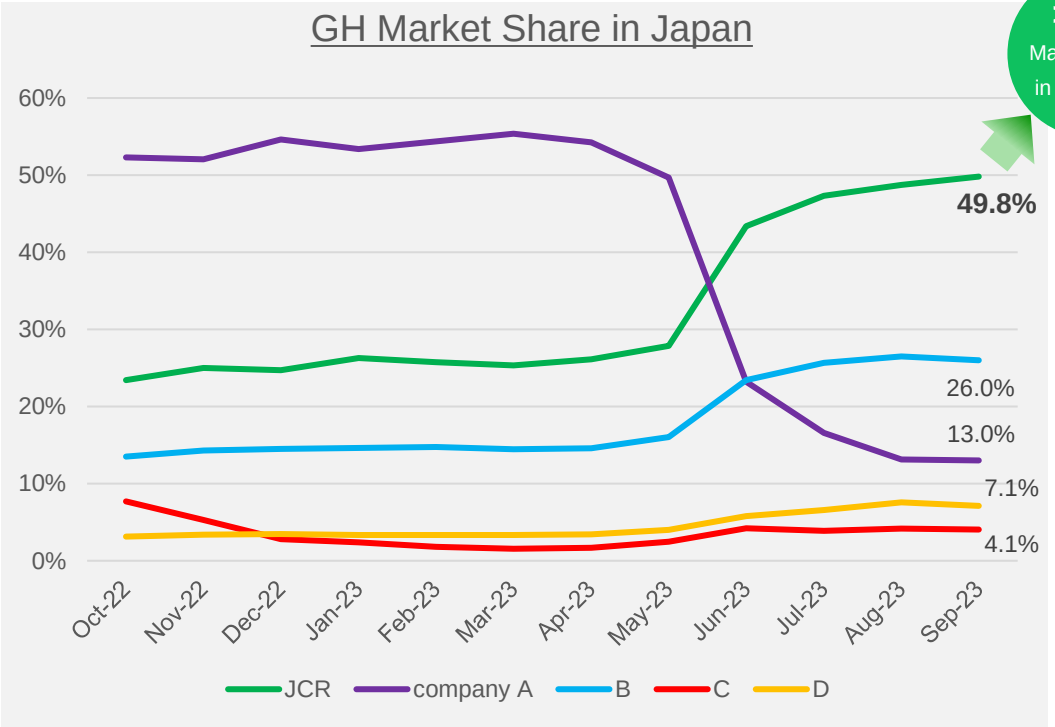
Demand for GROWJECT® has surged since May 2023.
JCR successfully achieved to increase production and stable supply of drug to patients.



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Own analysis based on JPM (Oct 2022-Sep 2023). Reprinted with permission

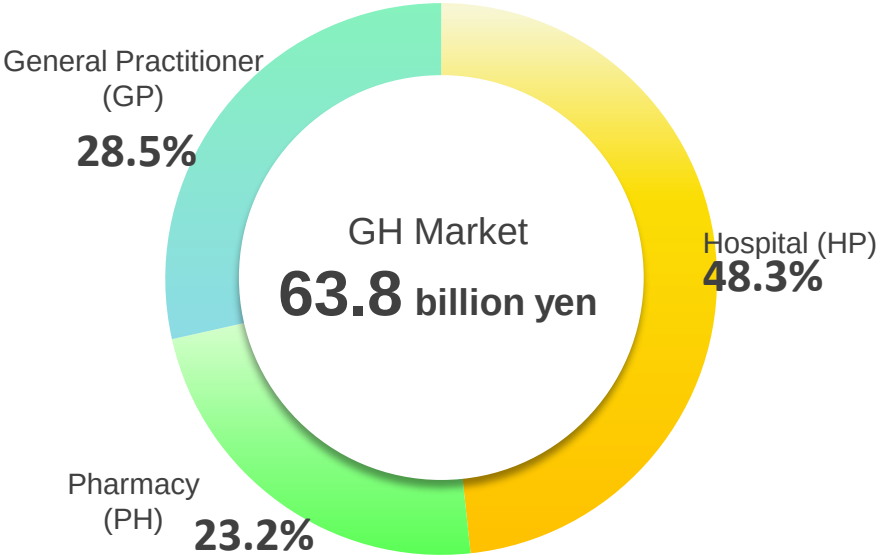
FY2023 1st-Half Results*

- Number of units shipped
  YoY approx. 1.6x
- Number of Naïve patients
  YoY approx. 1.8x
- Number of switched patients
  YoY 90 times more

*JCR internal analysis

Analysis of the Domestic GH Market*

Domestic GH market*



Hospital Market Characteristics

- More opportunities for SGA, Turner and SHOX with higher-dose per weight.
- Hospital specialists serve a KOLs to influence the entire healthcare community.
- Practices for drug management at hospitals make it difficult to adopt new drugs.

GROWJECT® Took the Top Market Share for All Sectors

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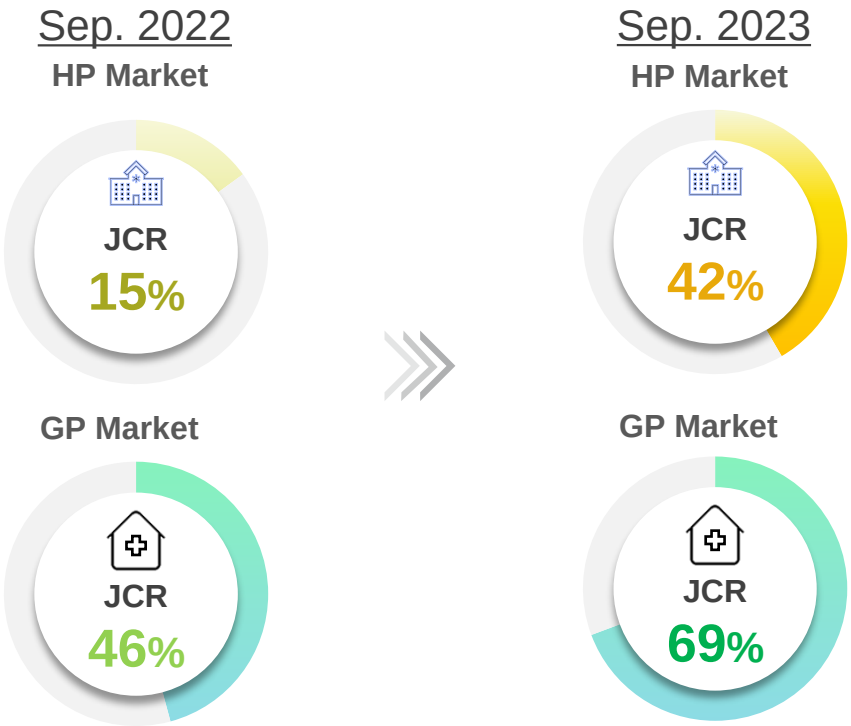
Significant expansion in the HP market, a long-standing challenge.



Number of facilities supplied with GROWJECT®*

(Compared to FY2022)

- HP：approx. 1.2x
  - New accounts ：180
- GP：approx. 1.4x
  - New accounts ：69

*Internal data

GROWJECT® HP Market Sales Ratio
GROWJECT® GP Market Sales Ratio
GH Sales Ratio excluding GROWJECT®

Our Sales Strategy for Further Growth

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Strong and stable supply

- Already implemented a system to secure the production volumes necessary to maintain a market share of more than 50%
- Continued stable supply as GH top share manufacturer and increased market confidence

Device strategy

Two types of devices to meet the needs of patients and healthcare professionals

Growjector® L

- Full support for injections
- Enhanced compliance through fun features

Growjector® DUO

- Easier operation mode reduces reluctance to switching devices from other devices
- Reduced injection teaching time at the time of prescribing

Entering more new patients and sustaining existing patients

Motorized Digital Injector “GROWJECTOR®”

Providing motorized digital injectors that suit the patient's needs/lifestyle

Main features of motorized digital injector：
Constant speed of drug infusion, pre-setting and automatic calculations of doses, automatic adjustment of doses during drug changes, recording of injection history, etc.

Expanding options in various treatment opportunities

Growjector® L

Operation guidance
Automatic needle injection/removal
Fun features
Dosage setting by Dr.

Friendly

Target： younger children

Growjector® Duo

Simple operation via home screen
Automatic injection (skin sensor)
Can be set without remote control
Can be stored in refrigerator

Simple

Target： older children and adult such as caregiver

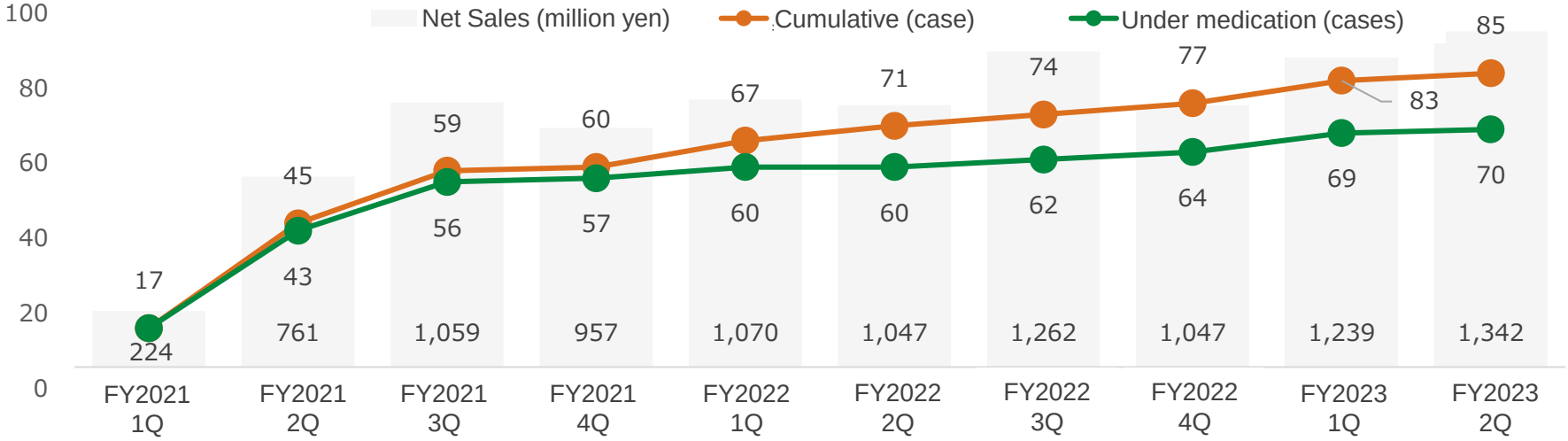
めろん日記

Management treatment records with an app

Connectivity

ⓘ Treatment for short stature
Treatment continues from infancy through school age to adolescence. Injections are administered primarily by parents when patients are young, and by the patients themselves as they get older.

IZCARGO® Prescription Status



Promotional Structure from Apr. 2023

- Promotion by IZCARGO® MR
- Co-promotion with Sumitomo Pharma Co., Ltd.

Accelerate sales in the current fiscal year by strengthening outreach and information-gathering capabilities

Est. Domestic Market Size (JCR analysis)

- **Patient Population: approx. 170**
- **Annual treatments Costs: 70-80 million yen per patient** (based on 30 kg bodyweight)

ⓘ ERT

ERT is the replacement of deficient enzyme by administering recombinant enzyme via i.v. infusion. It needs to continue throughout life.

ⓘ Dosage of IZCARGO®

2.0 mg/kg/w i.v. infusion.

TEMCELL® Prescription Status

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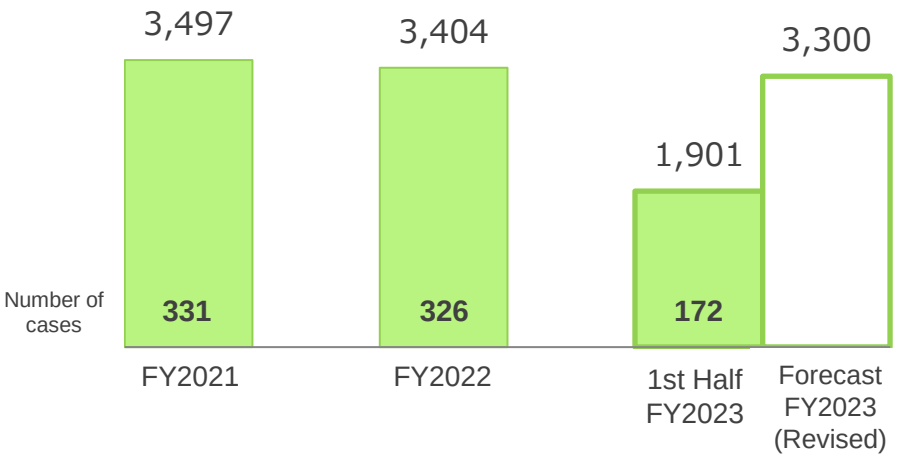
Sales trends (Unit: million yen)



Patient incidence (JCR analysis)

Onset of acute GVHD：in approx. 30% of HSCT patients

No response to steroid treatment：**approx. 35% of pts with aGVHD**

Amenable for TEMCELL®

TEMCELL® sales status update

- **Available in more than 90% of target facilities**
- With the cooperation of MEDICEO CORPORATION, the lead time was reduced from 3 to 2 days.
- Changes in the prevention and treatment of aGVHD: Impact currently is not significant.
  - —24, Jul -2023：
    Cyclophosphamide hydrate is covered by insurance for the prevention of GVHD.
  - —23, Aug-2023：
    Ruxolitinib Phosphate has been approved for the treatment of GVHD after HSCT that is unresponsive to steroids.

JCR Activities for Further Growth
2. R&D

Mathias Schmidt,PD,Ph.D.
Vice President,
Clinical development (overall supervision)
Business development and IR fields, excluding Japan

R&D Pipeline

★ Updated

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Code	Indication	Status	Upcoming Milestones
JR-141	MPS II (Hunter syndrome)	Global Ph3	~FY2027: Approval in US, EU, Brazil
JR-171	MPS I (Hurler syndrome etc.)	Global Ph1/2 completed	FY2024: Ph3
JR-441	MPS IIIA (Sanfilippo syndrome type A)	★ Global Ph1/2	★ 1st Half FY2024: LPI
JR-446	MPS IIIB (Sanfilippo syndrome type B)	Pre-clinical	FY2024: Ph1/2
JR-479	GM2 Gangliosidosis (Sandhoff, Tay-Sachs disease)	Pre-clinical	~FY2025: Ph1
JR-471	Fucosidosis	Pre-clinical	TBD
JR-162	Pompe disease	Pre-clinical	TBD
JR-443	MPS VII (Sly syndrome)	Pre-clinical	TBD
JR-142	Pediatric GHD	Ph2 (Analysis completed)	★ FY2024: Ph3 (Timing reviewed due to adjustments to the investigational drug production schedule)
JR-031HIE	Hypoxic ischemic encephalopathy in neonates	Ph1/2 (Analysis completed)	★ TBD（Phase 3 under consideration）

LSD Pipeline using J-Brain Cargo® Technology

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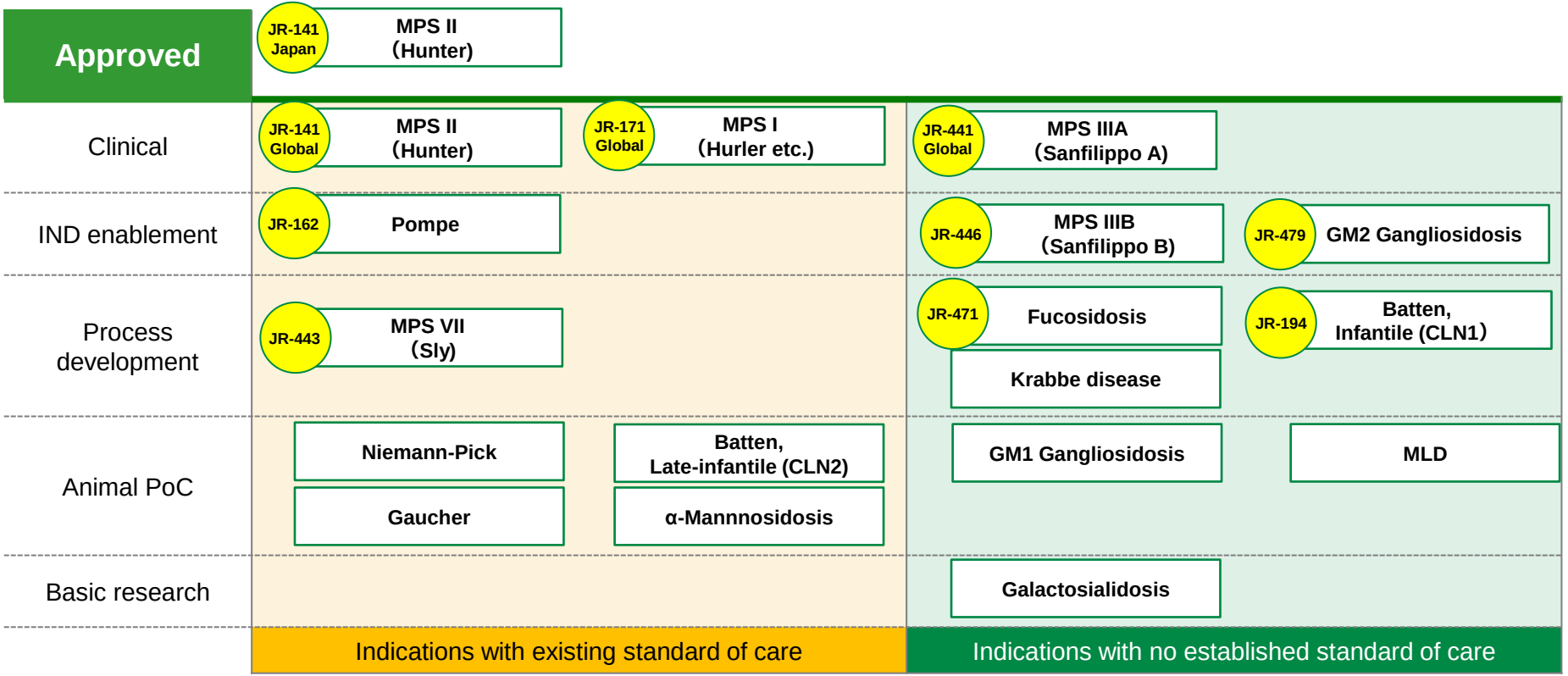
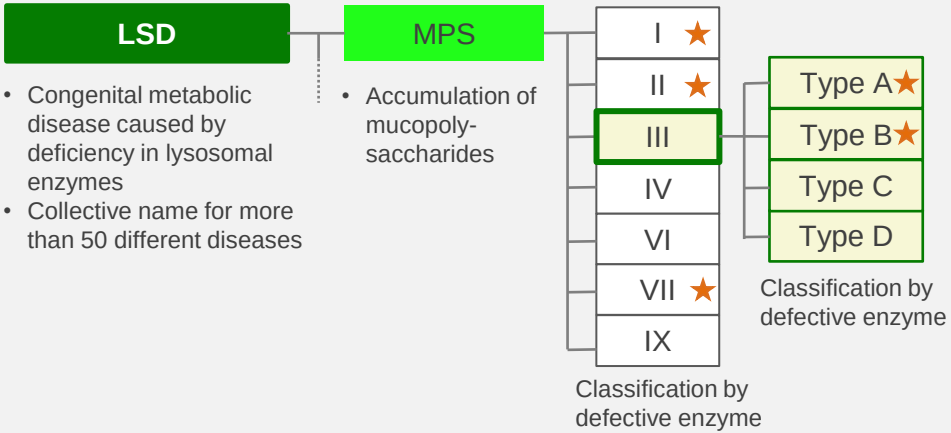
Built an industry-leading portfolio in the LSD space of over $10billion.



MPS III　Disease Overview



- Congenital metabolic disease caused by deficiency in lysosomal enzymes
- Collective name for more than 50 different diseases

- Accumulation of mucopoly-saccharides

MPS III

- Autosomal recessive disorder causing accumulation of toxic heparan sulfate in several tissues and organs
- CNS signs and symptoms are most prevalent, requiring enzyme replacement therapy to cross the blood-brain-barrier
- Type A is the most progressive and most prevalent.

Standard of Care

No established SoC:

- CNS symptoms only addressable with a BBB-penetrating therapy.
- Recombinant enzymes are particularly difficult to manufacture.

Est. Patient Population

	Japan	Worldwide
MPS IIIA	Under 10	1,000 -2,000
MPS IIIB	Est. 20	500 -1,000

Est. Market Size（JCR analysis）

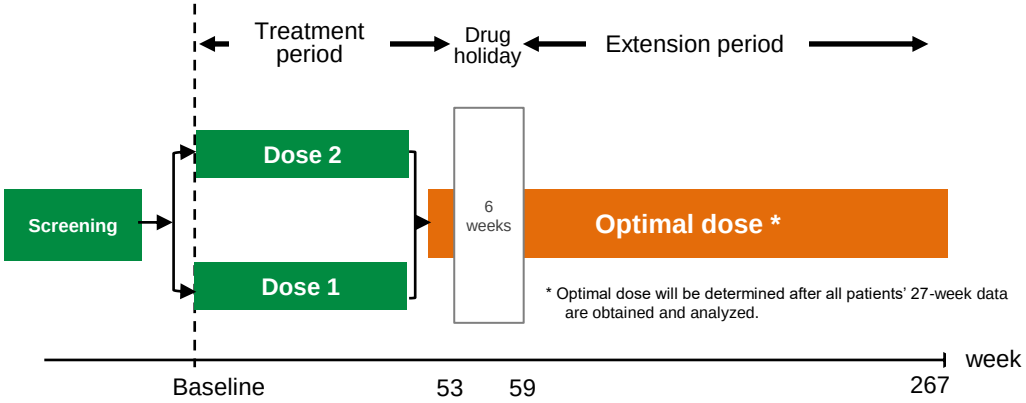
	Est. Annual Sales
MPS IIIA	60.0 billion yen
MPS IIIB	25.0 billion yen

- High risk of underdiagnosed disease due to lack of SoC.
- Absence of SoC promoted regulatory cooperation: Guidance issued by the FDA for the development of new treatments for MPS III (Feb- 2020).

★ Diseases with active programs at JCR

July 2023 - Approval of Global Phase I/II study (JR-441-101)

JR-441-101 study overview



Achievements and next milestones

- **Jan -2022**
  EC grants Orphan Drug Designation
- **Jul -2023**
  Approval of Global Ph I/II Clinical Trial in Germany
- **Oct -2023**
  First Patient First dosed
- **1st Half –FY2024**
  Last Patient
- **2nd Half –FY2025**
  1-year clinical data is expected

Overview	
Objectives	Safety, dose finding, exploratory efficacy
No. of subjects	12 subjects (Both rapidly progressing and slowly progressing, ≥1 year and ≤18 years)
Clinical Trails.gov	Identifer : NCT06095388

Market Potential (JCR analysis)

- **Est. Patient population**
  Japan: <10
  WW: 1,000-2,000
- **Est. Market size**
  >60.0 billion yen

JR-446（BBB-penetrating ERT for MPS IIIB)

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Sep-2023 MEDIPAL HOLDINGS and JCR Conclude Two Agreements on JR-446

MEDIPAL

	Japan	Rest of World
Agreement	**Collaboration agreement**	**Licensing agreement**
MA holder	JCR (jointly developed with MEDIPAL HD)	MEDIPAL HD (partially outsourced operations to other parties as required)
Est. number or patients	Approx. 20	500-1,000
Market size	1.0-2.0 billion yen	Approx. 25.0 billion yen

JR-446 Status

- Established molecular design, optimized for activity and manufacturability
- Currently in IND enablement
- Clinical trial with JR-446 to begin in 1st Half -FY2024

- Patient pool in Japan is sufficient to allow domestic development.
- Enhancing disease awareness and early screening will likely increase prevalence and incidence of MPS IIIB in any country.

Both companies will join forces and employ the most expeditious pathways toward approval in each geography.

ⓘ Partnering with MEDIPAL HD

Oct-2022: Commencement of efforts for global commercialization targeting Ultra-Rare diseases. Conclude a licensing for JR-471, treatment for Fucosidosis

JR-141(pabinafusp alfa: BBB-penetrating ERT for MPS II)

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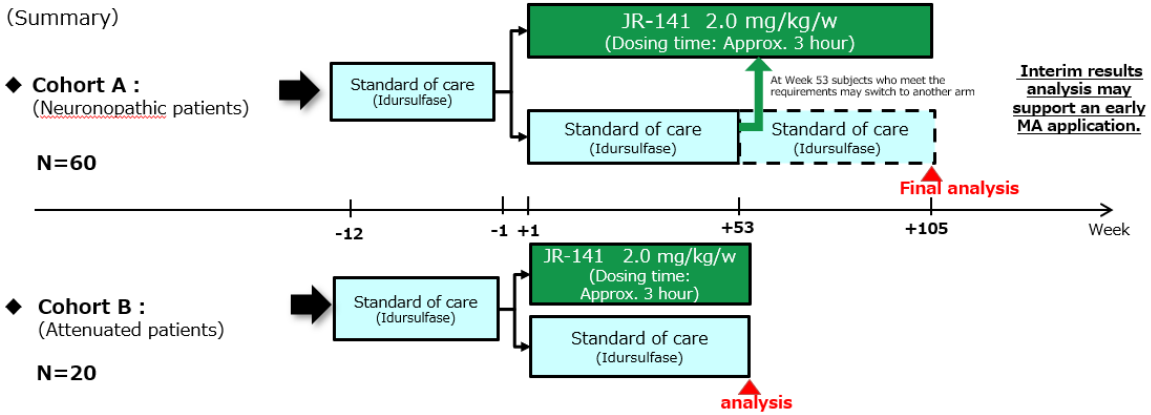
Global Phase III study（JR-141-GS31): STARLIGHT study Overview



Overview	
Objectives	1. To assess the efficacy of JR-141 on CNS signs and symptoms in MPS-II subjects relative to standard ERT 2. To assess control of somatic signs and symptoms by JR-141 relative to standard ERT
Endpoints	• Changes in HS in CSF, CNS symptoms (cognitive, behavior, attention) • Control of systemic sign and symptoms
Clinical Trails.gov	Identifier : NCT04573023

JCR Pharmaceuticals × Takeda

Upcoming milestone
1QFY2024 - all patients enrolled necessary for interim analysis

Current Status

- Recruiting
- Number of Clinical trial sites (as of Oct 2023):
  - USA: 5, Europe: 10, Brazil: 2
  - Further sites to open in EU, USA, LATAM and Asia to accelerate recruitment

Achievements

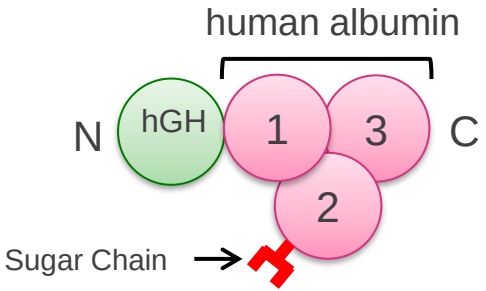
- **Oct -2018** ODD by FDA
- **Feb -2019** ODD by EMA
- **Feb -2021** Fast Track Designation by FDA
- **Oct -2021** PRIME Designation by EMA
- **Feb -2022** First Patient dosed in JR-141-GS31

JR-142（Long-acting GH）

The growth promoting effect of JR-142 was confirmed in Phase II trial.
Phase III trial scheduled to start in FY2024.

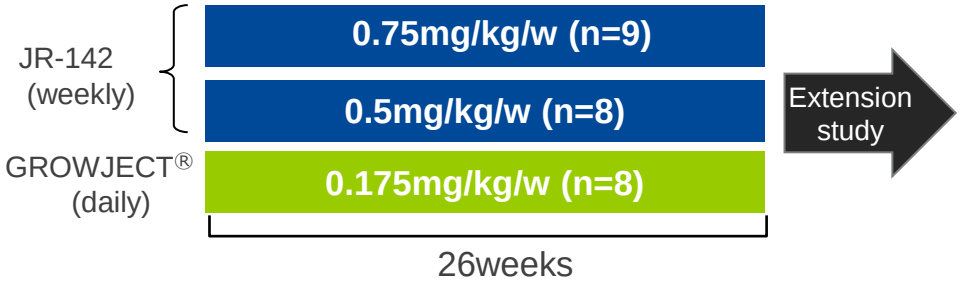
JR-142 molecular design

Modified albumin-fused GH



In-house development of fusion protein with modified albumin glycosylation to improve blood retention.

Phase 2 study design



Subjects	Pediatric growth hormone deficiency
Endpoints	• PK/PD • Changes in Height velocity • Safety
Details	jRCT(Identifier : jRCT2031200372)

ⓘ Growth hormone treatment in Japan
Daily GH (5 products), Weekly GH (2 prodcuts)

JR-142 Ph2 results: Comparable Safety and Efficacy Profile

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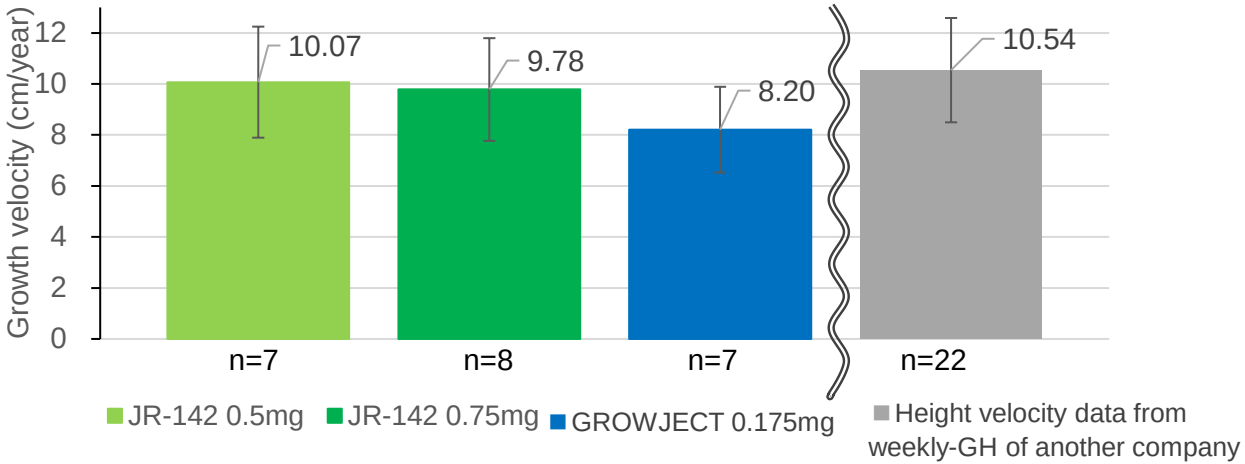
Key outcomes:

- Height velocity: Comparable between GROWJECT® and JR-142.
- AEs/safety profile: Comparable between all groups.

Outcome of Phase 2 Trial (height velocities)



Next milestones

- 2nd-Half FY2024
  Start Ph3
- FY2027
  File for Marketing authorization

Domestic Market Size

- approx. 18% of GH market share
  (as of Sep. 2023; JCR analysis)

Reference: List of growth rates after GH treatment
(drawn in-house from internal data, published materials, etc.)

Other Activities to enhance Access to Medicine

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- JR-031HIE: Expanded indication of TEMCELL® HS inj. for Hypoxic ischemic encephalopathy in neonates
  (detail: jRCT1080224818)
  - Future development plan under consideration based on results of Ph I/II and 18-month observational studies.
    - Comparative study of hypothermia alone (7 subjects) and TEMCELL® plus hypothermia (6 subjects)
    - Over 65% of subjects in both groups were found to benefit from treatment after 18 months of treatment. However, no differences between groups were observed.
    - No safety issues were identified.

- Cooperation in investigator-initiated clinical trials
  - Provided investigational drug for "Phase II/III open study to evaluate the efficacy and safety of chaperone therapy with ambroxol hydrochloride (JT408T) in patients with neuronopathic Gaucher disease”

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Appendix

Highlights（May 11, 2023- Oct. 27, 2023）

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JCR Pharmaceuticals

Category	Date	News
R&D	11 May	Novel Biologic Therapies in Epilepsy　Collaboration with Angelini Pharma
Sustainability	14 May	LSD-related webinars for the general public
Core products	23 May	IZCARGO® Add of injectable drugs that can be administered by insurers to covered drugs
Company	5 Jun	Receives the 48th Inoue Harushige Prize for Therapeutics for LSDs with an Original BBB Penetrating Technology
Core products	26 Jun	Approval for Expanded Indication of GROWJECT® in Short Stature due to SHOX-Deficiency
R&D	11 Jul	JR-441　Approval to Start Global Ph I/II Clinical Trial in Germany
Company	14 Aug	Receipt of 2023 Kinki Branch Chief's Award for Outstanding Contribution to Electrical Safety from Chubu Kinki Industrial Safety and Inspection Department
Core products	29 Aug	GROWJECTOR®D u o launched on 1 Sep.
Sustainability	31 Aug	Sponsorship for Relay for Life Japan
Company	7 Sep	Recognition in the 2nd Hyogo Kobe Women's Empowerment Business (Mimoza Certified Company) Accreditation
Company	28 Sep	Upward Revision of Consolidated Financial Forecasts for FY2023
R&D	28 Sep	Conclude two agreements on MPS IIIB with MEDIPAL HOLDINGS
Core products	2 Oct	Upgrade of Melon Nikki™
R&D	4 Oct	JR-171 52-week interim data meeting
R&D	27 Oct	JR-441-101 study FPI

Angelini Pharma

PHC Healthcare with Precision

ひょうご・こうべ 女性活躍推進認定 ミモザ企業

MEDIPAL

PHC Healthcare with Precision

Product history of GROWJECT® administration device

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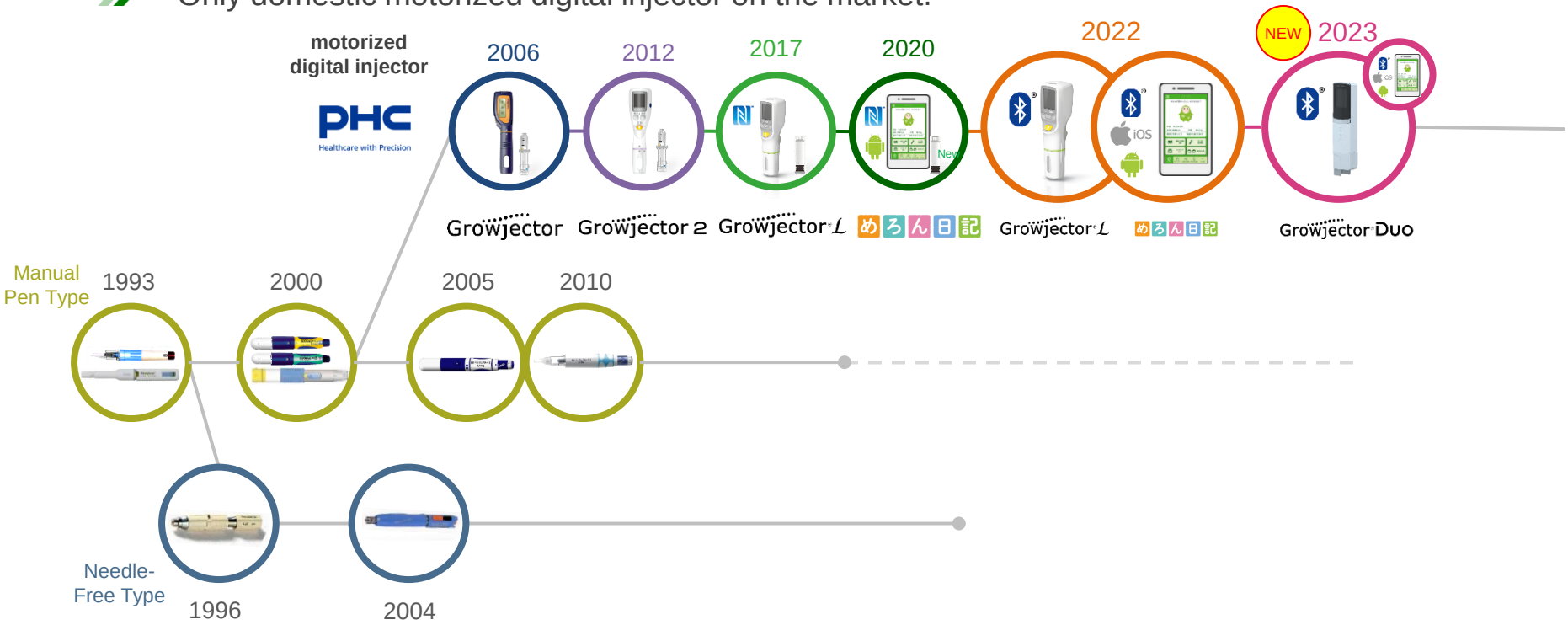
Improved devices, a key factor in prescribing, at an unparalleled pace.

- As the only domestic manufacturer, continues to develop devices that meet the needs of healthcare professionals and patients in Japan.
- Only domestic motorized digital injector on the market.



Pipeline of LSDs

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

Indication :	MPS type II (Hunter syndrome)
Patient population*1 :	150-200 (Japan) ,2,000-3,000 (WW) est.
Est. Market size*2 :	8.0-10.0 billion JPY (Japan), 90.0 billion JPY (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 :	70 (Japan), 3,000-4,000 (WW) est.
Est. Market size*2 :	2.0-3.0 billion JPY (Japan), 60.0 billion JPY (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

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

Indication :	MPS type III A (Sanfilippo A syndrome)
Patient population*1 :	10 (Japan) , 1,000-2,000 (WW) est.
Est. Market size*2 :	1.0-2.0 billion JPY (Japan), 60.0 billion JPY (WW)
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 :	100-150 (Japan), 10,000 (WW) est.
Est. Market size*2 :	3.0 billion JPY (Japan), 160 billion JPY (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

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

Indication :	MPS type VII（Sly syndrome）
Patient population*1 :	<10 (Japan) , 100-200 (WW) est.
Est. Market size*2 :	3.0 billion JPY (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 :	20 (Japan）, 500-1,000 (WW) est.
Est. Market size*2 :	1.0-2.0 billion JPY (Japan), 25.0 billion JPY (WW)
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

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

Indication :	GM2 gangliosidosis (Tay-Sachs disease, Sandohoff disease)
Patient population*1 :	20 (Japan), 1,000-2,000(WW) est.
Est. Market size*2 :	2.0-3.0 billion JPY (Japan), 55.0 billion JPY (WW)
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.

JR-471 BBB-penetrating α-L-fucosidase (rDNA origin)

Indication :	Fucosidosis
Patient population*1 :	<10 (Japan) , 100-200 (WW) est.
Est. Market size*2 :	<1.0 billion JPY (Japan), 15.0 billion JPY (WW)
Disease overview :	Fucosidosis is an autosomal recessive LSD caused by a deficiency in the glycoprotein-metabolizing enzyme (α-L-fucosidase) . Symptoms include psychomotor symptoms, muscle hypotonia, visceromegaly, and skeletal abnormalities. The disease can be classified in the rapidly progressive form, causing severe, life-threatening complications in children or in the mild form develop during adolescence and with slower progression, but causing serious complications in adulthood.

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

Pipeline of Other Compounds

Reach Beyond, Together 一緒に、その先へ

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）

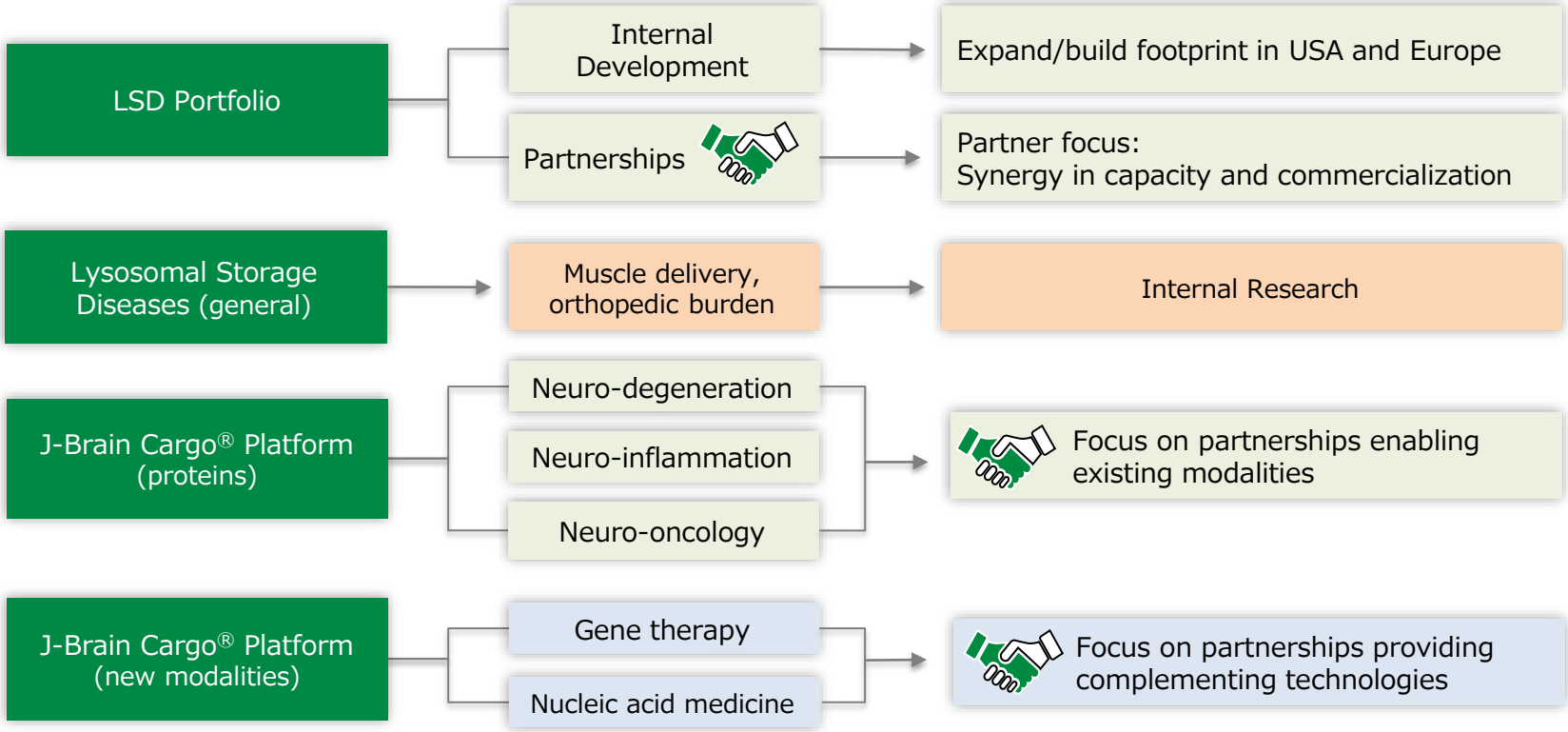
JR-031HIE Human mesenchymal stem cells（Expanded indication of TEMCELL®HS Inj.）

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

Leveraging Growth Opportunities from J-Brain Cargo®

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



Abbreviations （A～G）

AE	Adverse Events	有害事象
ANVISA	Brazilian Health Surveillance Agency	ブラジル国家衛生監督庁
BBB	Blood-Brain Barrier	血液脳関門
CDMO	Contract Development and Manufacturing Organization	医薬品開発製造受託機関
CNS	Central Nervous System	中枢神経系
CSF	Cerebrospinal fluid	脳脊髄液
DP	Darbepoetin Alfa BS Inj. [JCR]	ダルベポエチン アルファBS注「JCR」
DQ	Development Quoitent	発達指数
DS	Dermatan Sulfate	デルマタン硫酸
EC	European Commission	欧州委員会
EMA	European Medicines Agency	欧州医薬品庁
EPO	Epoetin Alfa BS Inj. [JCR]	エポエチンアルファBS注「JCR」
ERT	Enzyme Replacement Therapy	酵素補充療法
FDA	Food and Drug Administraion	米国食品医薬品局
GH	Growth Hormone	成長ホルモン
GHD	Growth Hormone Deficiency	成長ホルモン分泌不全性低身長症
GVHD	Graft versus Host Disease	移植片対宿主病

Abbreviations （H～Z）

HIE	Hypoxic ischemic encephalopathy in neonates	低酸素性虚血性脳症
HS	Heparan Sulfate	ヘパラン硫酸
HSCT	Hematopoietic Stem Cell Transplantation	造血幹細胞移植
i.v.	Intravenous Injection	静脈注射
IAR	Infusion-associated reactions	投与時反応
JBC	J-Brain Cargo® Technology	J-Brain Cargo®技術
KOL	Key Opinion Leader	影響力を持つ専門家
LSD	Lysosomal Storage Disease	ライソゾーム病
M6PR	Mannnose-6-phosphate receptor	マンノース6リン酸受容体
MPS	Mucopolysaccharidosis	ムコ多糖症
MSC	Messenchymal Stem Cell	間葉系幹細胞
PD	Pharmacodynamics	薬力学
Ph I	Phase I	臨床第I相試験
Ph II	Phase II	臨床第II相試験
Ph III	Phase III	臨床第III相試験
PK	Pharmacokinetics	薬物動態
PMDA	Pharmaceuticals and Medical Devices Agency	独立行政法人医薬品医療機器総合機構
R&D	Research and Development	研究開発
RD	Rare Disease	希少疾病
TfR	Transferrin Receptor	トランスフェリンレセプター