

# Appendix to the Consolidated Financial Summary FY2023 3rd Quarter

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January 26, 2024

JCR Pharmaceuticals Co., Ltd.

【Securities code】 TSE 4552

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# Consolidated Financial Results



(Unit: million yen)

Consolidated	FY2022		FY2023				
	Full-year results	Q3 YTD results	Q3 YTD results	Year-on-year		Full-year forecast (Revised)	Progress rate
				Difference	Ratio		
Net sales	34,343	26,696	33,718	+7,022	+26.3%	45,400	74.3%
Cost of sales	8,886	6,899	8,423	+1,523	+22.1%	12,400	67.9%
Gross profit	25,456	19,796	25,295	+5,498	+27.8%	33,000	76.7%
Selling, general and administrative expenses	20,480	14,843	17,486	+2,642	+17.8%	22,500	77.7%
SG&A	11,678	8,426	9,127	+701	+8.3%	12,800	71.3%
R&D expenses	8,802	6,417	8,359	+1,941	+30.3%	9,700	86.2%
Operating profit	4,975	4,953	7,809	+2,855	+57.7%	10,500	74.4%
Non-operating income	541	414	575	+160	+38.7%	-	-
Non-operating expenses	99	76	995	+919	+1206.6%	-	-
Ordinary profit	5,418	5,291	7,388	+2,097	+39.6%	10,000	73.9%
Extraordinary income	10	-	0	+0	-	-	-
Extraordinary losses	16	11	18	+6	+61.9%	-	-
Profit before income taxes	5,412	5,280	7,371	+2,090	+39.6%	-	-
Income taxes	1,639	1,711	2,210	+498	+29.2%	-	-
Profit attributable to owners of parent/Profit	3,772	3,568	5,160	+1,591	+44.6%	7,300	70.7%

# Consolidated Financial Results



- **Sales and profits increased significantly** year on year, primarily due to increase in product sales and Income from contractual payments.
- **Cost of sales** were below the internal forecast, with the cost of sales ratio showing an improving trend.
- **Selling, general and administrative expenses** were below the internal forecast due to efficient business execution.
- **R&D expenses** were above the internal forecast. This was mainly due to operating expenses incurred in an acceleration of global clinical trials.
- Share of loss of entities accounted for using equity method related to Mycenax Biotech Inc. in Taiwan and AlliedCel Corporation, which are equity-method affiliates, were recorded as **non-operating expenses**.
- There has been no change in the effective tax rate for **income taxes** since the previous fiscal year.

(Unit: number of people)

Changes in the Number of Employees	As of December 31, 2022	As of December 31, 2023	Increase
Non-consolidated	834	900	66
Consolidated	851	932	81

# Breakdown of Sales of Pharmaceuticals, etc. (Consolidated)



(Unit: million yen)

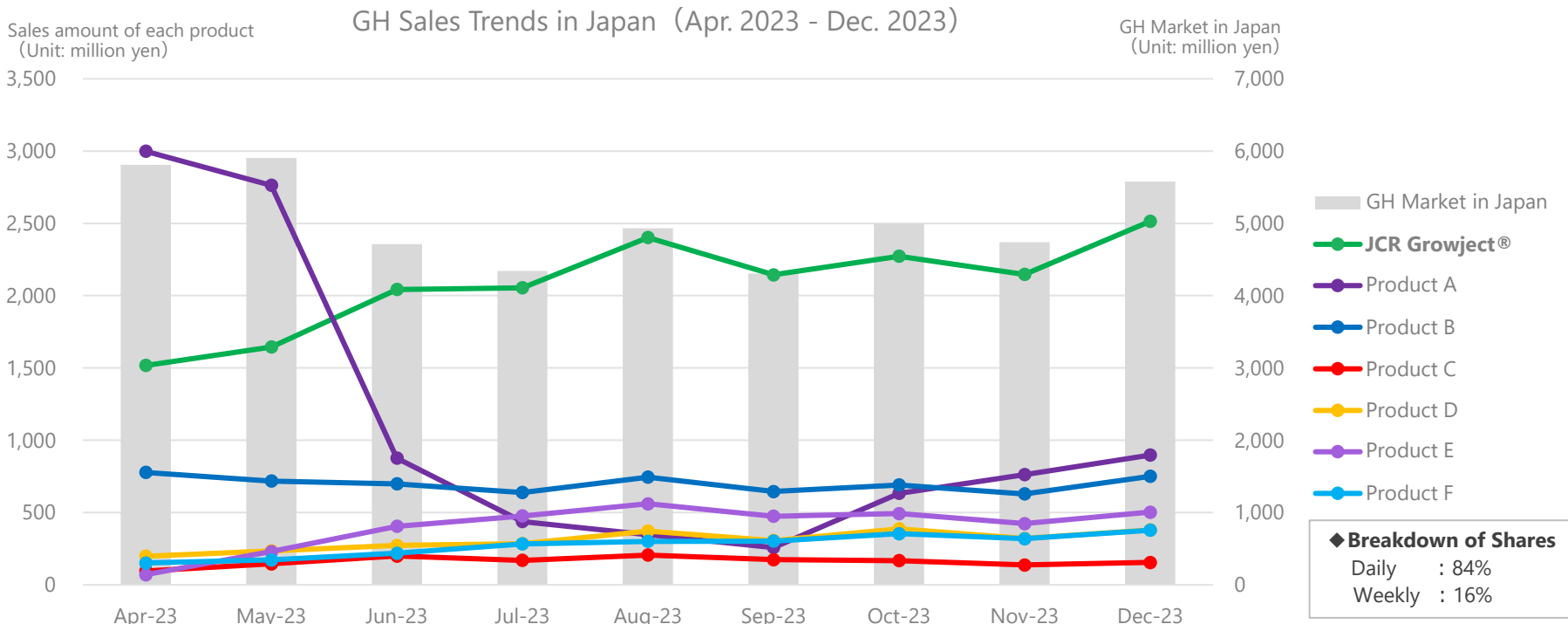
Consolidated	FY2022		FY2023				
	Full-year results	Q3 YTD results	Q3 YTD results	Year-on-year		Full-year forecast (Revised)	Progress rate
				Difference	Ratio		
<b>GROWJECT®</b>	12,261	9,320	<b>13,995</b>	+4,674	+50.1%	19,500	<b>71.8%</b>
<b>IZCARGO®</b>	4,428	3,380	<b>4,046</b>	+666	+19.7%	5,500	<b>73.6%</b>
<b>TEMCELL® HS Inj.</b>	3,404	2,560	<b>2,699</b>	+138	+5.4%	3,300	<b>81.8%</b>
<b>Treatment for renal anemia</b>	4,696	3,573	<b>3,673</b>	+99	+2.8%	5,000	<b>73.5%</b>
<b>Epoetin Alfa BS Inj. [JCR]</b>	2,710	2,084	<b>1,681</b>	(403)	(19.4)%	2,200	<b>76.4%</b>
<b>Darbepoetin Alfa BS Inj. [JCR]</b>	1,986	1,489	<b>1,992</b>	+503	+33.8%	2,800	<b>71.2%</b>
<b>Agalsidase Beta BS I.V. Infusion [JCR]</b>	964	835	<b>998</b>	+163	+19.5%	1,400	<b>71.3%</b>
<b>Total Pharmaceutical Products</b>	25,755	19,671	<b>25,413</b>	+5,742	+29.2%	34,700	<b>73.2%</b>

Sales of JCR's core products **GROWJECT®**, **IZCARGO®**, and **TEMCELL®** increased year on year. Sales of GROWJECT® and IZCARGO® progressed in line with internal forecasts, while TEMCELL® exceeded the internal forecast.

# GROWJECT® Market Trends

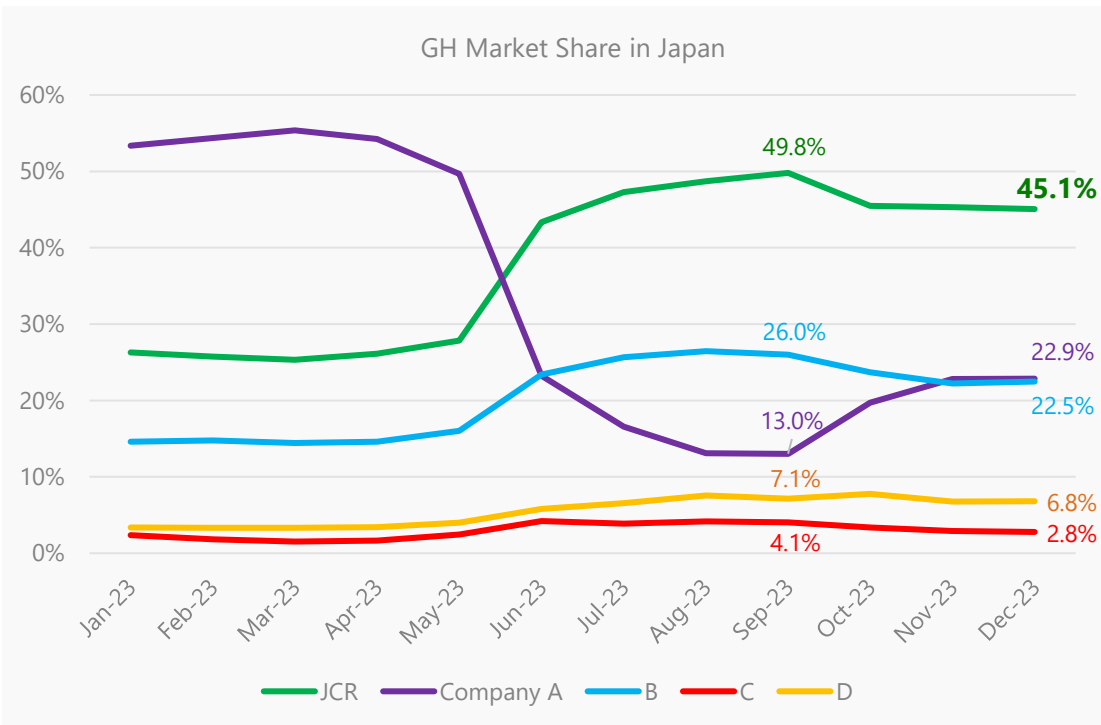


➤ Monthly sales reached a record high in December 2023 (2.51 billion yen) \*On an NHI drug price basis



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# GROWJECT® Market Trends



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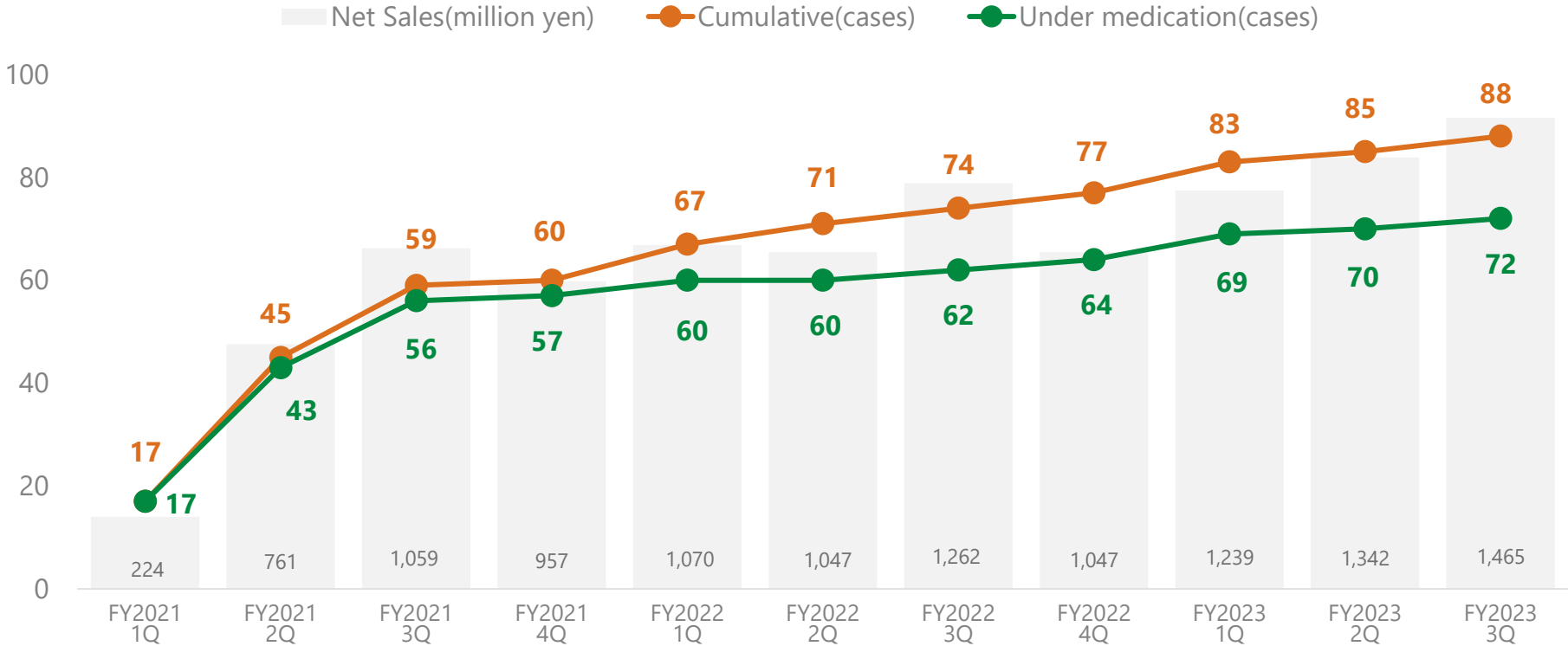
## ■ GROWJECT® Market Share by buyer

	Dec. 2023	Sales Change vs. Q2 <small>*On an NHI drug price basis</small>
HP Market	38%	+85 million yen
GP Market	63%	+155 million yen

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Market definition by JCR Reprinted with permission

HP: Hospital  
GP: General Practitioner

# IZCARGO® Prescription Status



# Breakdown of Net Sales (Consolidated)



(Unit: million yen)

Consolidated	FY2022		FY2023				
	Full-year results	Q3 YTD results	Q3 YTD results	Year-on-year		Full-year forecast (Revised)	Progress rate
				Difference	Ratio		
<b>Total Pharmaceutical Products</b>	25,755	19,671	<b>25,413</b>	+5,742	+29.2%	34,700	<b>73.2%</b>
<b>Income from contractual payment</b>	6,546	5,010	<b>7,112</b>	+2,102	+42.0%	8,100	<b>87.8%</b>
<b>Other</b>	109	83	<b>1,192</b>	+1,109	+1334.3%	2,600	<b>45.9%</b>
<b>AZD1222 stock solution</b>	1,931	1,931	-	-	(100.0%)	-	-
<b>Total net sales</b>	34,343	26,696	<b>33,718</b>	+7,022	+26.3%	45,400	<b>74.3%</b>

- For **Income from contractual payment**, contract negotiations continue to be carried out, to achieve the full-year forecast.
- Contract manufacturing sales recorded as **Other** is progressing as planned.
- The contract to manufacture AstraZeneca K.K.'s COVID-19 vaccine solution (AZD1222) in Japan was completed as planned in FY2022.





# R&D Pipeline



(Unit: million yen)

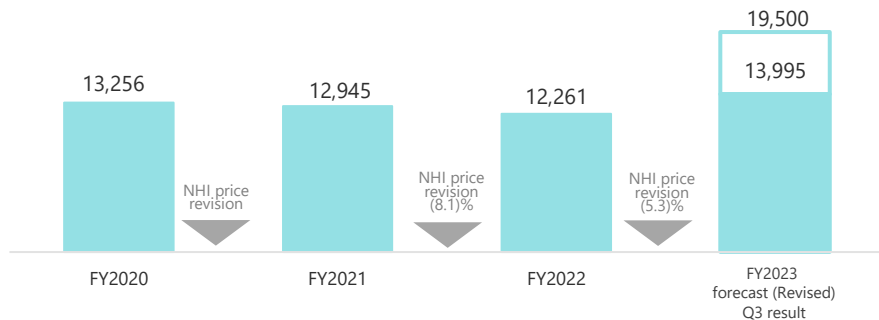
Consolidated	FY2022		FY2023				
	Full-year results	Q3 YTD results	Q3 YTD results	Year-on-year		Full-year forecast (Revised)	Progress rate
				Difference	Ratio		
R&D expenses	8,802	6,417	8,359	+1,941	+30.3%	9,700	86.2%
R&D expenses rate relative to sales	25.6%	24.0%	24.8%	-	-	21.4%	-

Code	Indication	Preclinical	Clinical	Application	Approved	Upcoming Milestones	
JR-141	MPS II (Hunter syndrome)	Global Ph3					Q1 FY2024: all patients enrolled necessary for interim analysis ~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 2nd Half FY2025: Analysis completed
JR-446	MPS IIIB (Sanfilippo syndrome type B )						FY2024: Ph1/2
JR-479	GM2 Gangliosidosis (Sandhoff, Tay-Sachs disease)						~FY2025: Ph1
JR-471	Fucosidosis						TBD
JR-162	Pompe disease						TBD
JR-443	MPS VII (Sly syndrome)						TBD
JR-142	Pediatric GHD	Ph2 (Analysis completed)					FY2024: Ph3
JR-031HIE	Hypoxic ischemic encephalopathy in neonates	Ph1/2 (Analysis completed)					TBD (Phase 3 under consideration)

# Net Sales Trends by Product



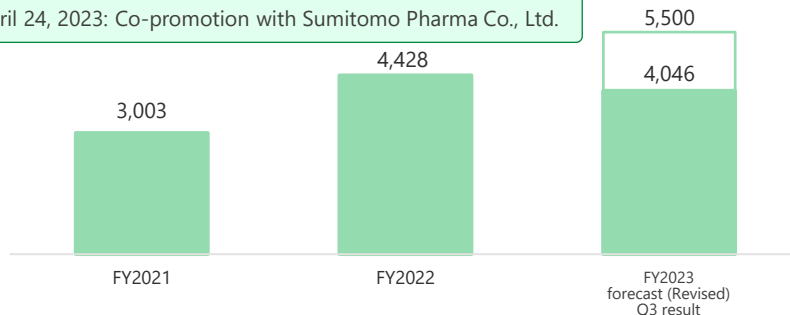
Recombinant human growth hormone product  
**GROWJECT®**



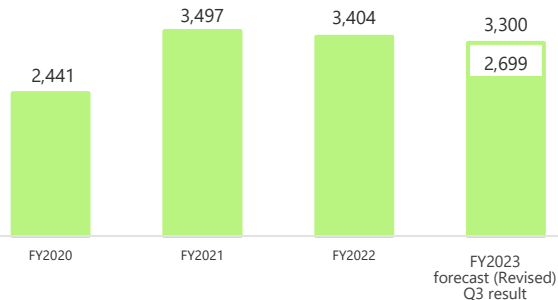
Recombinant therapeutic enzyme for mucopolysaccharidosis II (MPS II)  
**IZCARGO® I.V. infusion 10mg**

(Unit: million yen)

From April 24, 2023: Co-promotion with Sumitomo Pharma Co., Ltd.

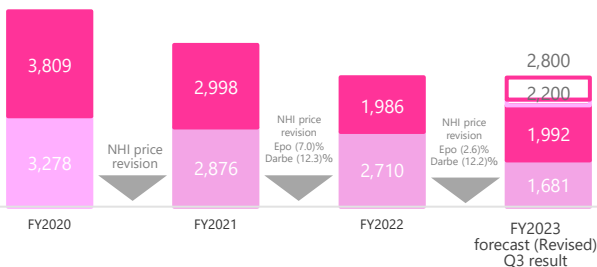


Human somatic stem cell-processed products  
Human (allogenic) bone marrow-derived mesenchymal stem cells  
**TEMCELL® HS Inj.**



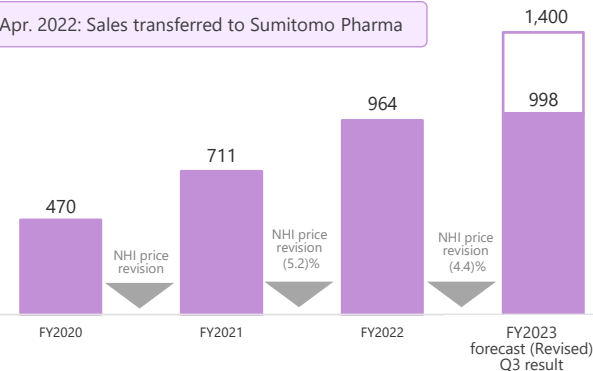
Recombinant erythropoietin product  
**Epoetin Alfa BS Inj. [JCR]**  
Long-acting erythropoiesis-stimulating agent  
**Darbepoetin Alfa BS Inj. [JCR]**

■ Epoetin Alfa  
■ Darbepoetin Alfa



Recombinant treatment for Fabry disease  
**Agalsidase Beta BS I.V. Infusion [JCR]**

Apr. 2022: Sales transferred to Sumitomo Pharma



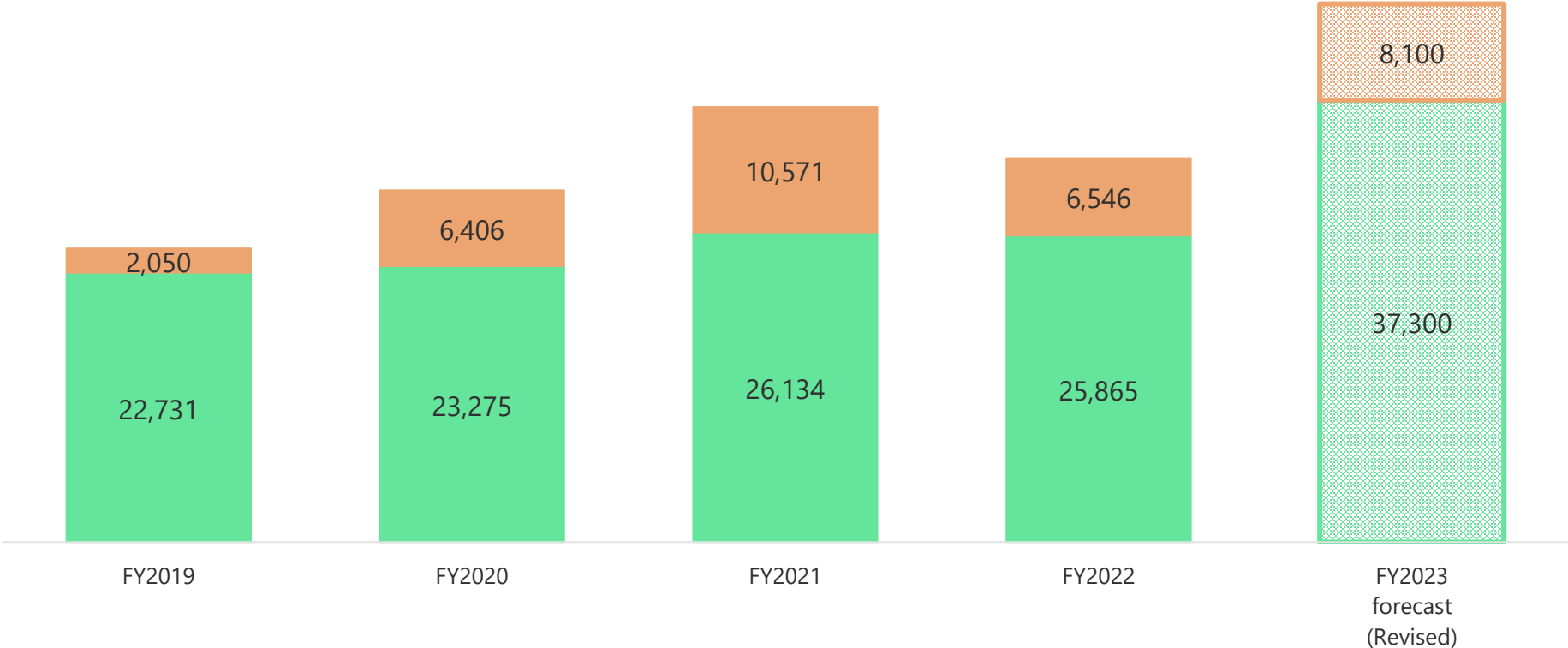
# Net Sales Trends (Excluding AZD1222 Stock Solution)



(Unit: million yen)

■ Products\* sales   ■ Income from contractual payment

\* Products: GROWJECT, IZCARGO, treatment for renal anemia, TEMCELL, Agalsidase Beta, others

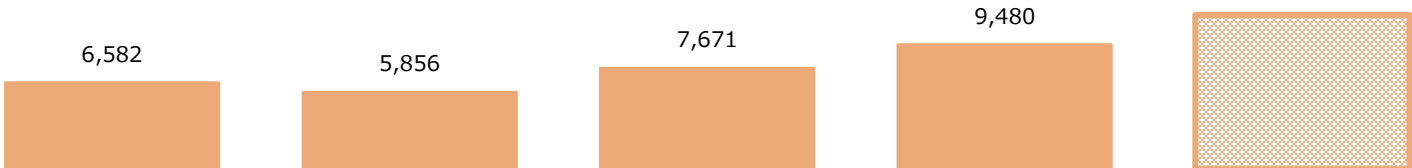


# Correlation between R&D Expenses (before Deducting) and Main R&D Pipeline



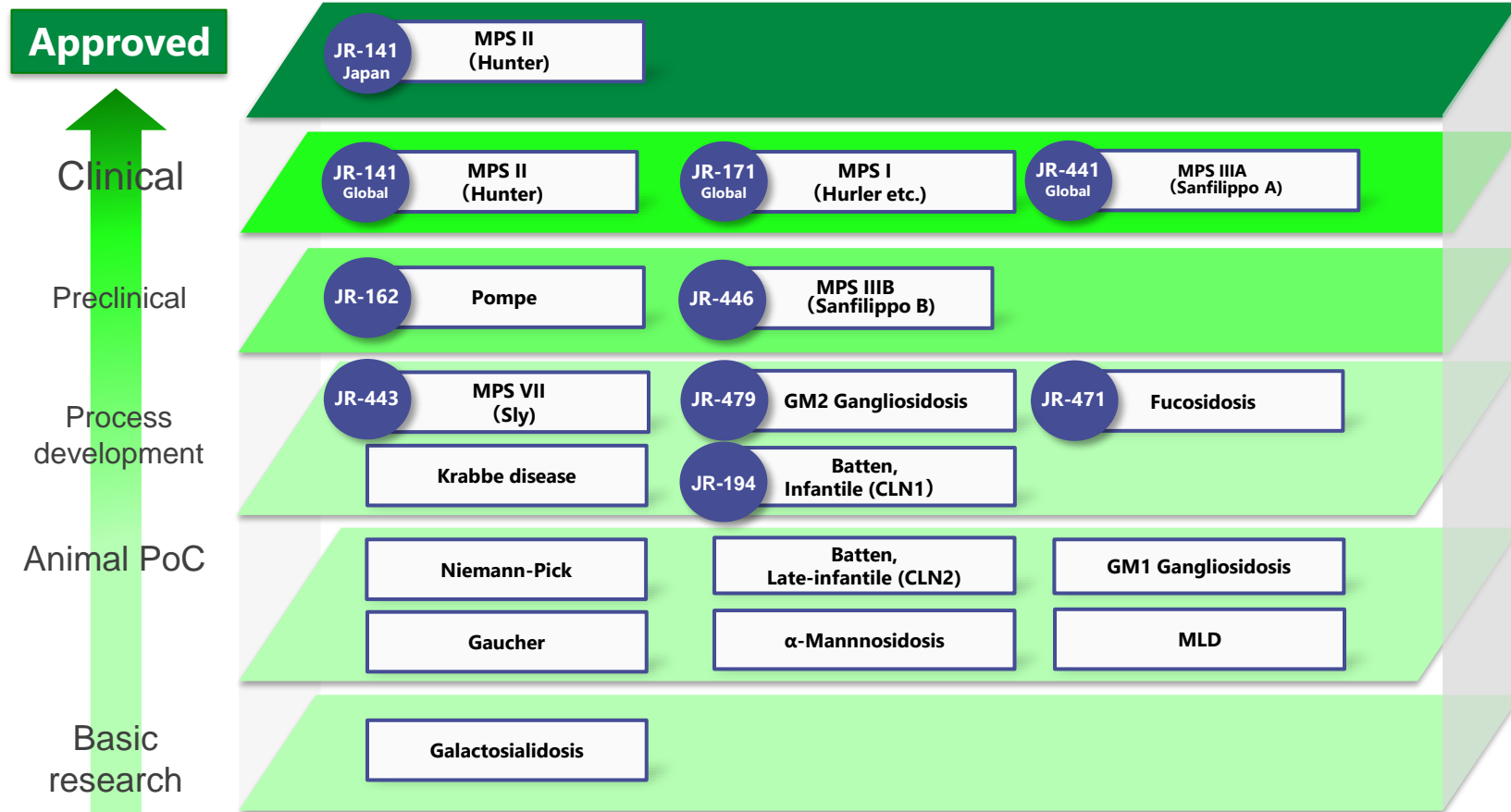
(Unit: million yen)

- Process development/  
Non-clinical
- Clinical trial
- ★ Filed an application
- Approved



	FY2019	FY2020	FY2021	FY2022	FY2023 (forecast)
JR-141 (Japan)		★ ○			
JR-141 (Brazil)		★		Aug. 2022: Approval denied	
JR-141 (Global)					
JR-171 (Global)					
JR-162					
JR-441					
JR-443					
JR-446					
JR-479					
JR-471					
JR-401X				★	○
JR-142					
JR-031HIE					
JTR-161/JR-161				Apr. 2022: Joint research completed	

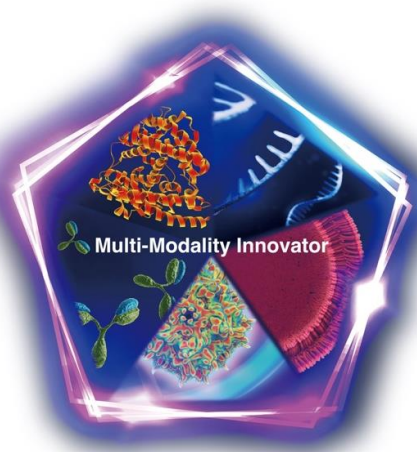
# LSD Pipeline using J-Brain Cargo® Technology



# Applicability of Platform Technology to Various Diseases

## Cutting-edge drug development following J-Brain Cargo<sup>®</sup> technology

The J-Brain Cargo<sup>®</sup> Platform reaches beyond the CNS



J-Brain Cargo<sup>®</sup>

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<sup>®</sup> (Japan)

Gene therapy Partnership with Takeda

JBC partnership with Alexion, Angelini

**ALEXION**  
AstraZeneca Rare Disease

**Angelini Pharma**

2020

2021

2022

2023

IZCARGO<sup>®</sup> partnership with Takeda



Ultra-rare diseases partnership with MEDIPAL



JBC partnership with Sumitomo Pharma (2015) Sumitomo Pharma

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## JR-141

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

<b>Indication :</b>	<b>MPS type II (Hunter syndrome)</b>
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  $\alpha$ -L-iduronidase (rDNA origin)

<b>Indication :</b>	<b>MPS type I (Hurler, Hurler-Scheie , Scheie syndrome)</b>
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 $\alpha$ -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 data from MHLW and own research \*2 Internal analysis



# Pipeline of LSDs



## JR-441

### BBB-penetrating heparan-N-sulfatase (rDNA origin)

<b>Indication :</b>	<b>MPS type III A (Sanfilippo A syndrome)</b>
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 $\alpha$ -glucosidase (rDNA origin)

<b>Indication :</b>	<b>Pompe disease</b>
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 $\alpha$ -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.

# Pipeline of LSDs



## JR-443 BBB-penetrating $\beta$ -glucuronidase (rDNA origin)

<b>Indication :</b>	<b>MPS type VII (Sly syndrome)</b>
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, $\beta$ -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 $\alpha$ -N-acetylglucosaminidase (rDNA origin)

<b>Indication :</b>	<b>MPS type III B (Sanfillipo B syndrome)</b>
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 $\alpha$ -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 data from MHLW and own research \*2 Internal analysis

# Pipeline of LSDs



## JR-479

BBB-penetrating  $\beta$ -Hexosaminidase A (rDNA origin)

<b>Indication :</b>	<b>GM2 gangliosidosis (Tay Sachs disease, Sandhoff disease)</b>
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 $\beta$ -Hexosaminidase A. GM2 ganglioside is abundant in the brain, and GM2 gangliosidosis gives rise to progressive CNS symptoms. It is difficult to distinguish between Tay-Sachs and Sandhoff disease by clinical symptoms.

## JR-471

BBB-penetrating  $\alpha$ -L-fucosidase (rDNA origin)

<b>Indication :</b>	<b>Fucosidosis</b>
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 ( $\alpha$ -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 data from MHLW and own research \*2 Internal analysis

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

<b>Indication :</b>	<b>Pediatric growth hormone deficiency</b>
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.)

<b>Indication :</b>	<b>Neonatal Hypoxic Ischemic Encephalopathy</b>
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