Reach Beyond, Together

FY2024 First Quarter Results Conference Call

July 30, 2024

JCR Pharmaceuticals Co., Ltd.

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Overview: Consolidated Financial Results

Reach Beyond, Together

(Unit : million yen)

Consolidated	FY2023	FY2024			
	Q1 YTD	Q1 YTD	Year-on-year		Progress Rate
			Difference	Rate of change	
Net Sales	10,808	**8,145**	(2,663)	(24.6%)	19.7%
Cost of Sales	3,363	**2,073**	(1,289)	(38.4%)	19.9%
Gross Profit	7,445	**6,072**	(1,373)	(18.4%)	19.7%
Selling, General and Administrative Expenses	5,379	**6,368**	+989	+18.4%	25.0%
SG&A Expenses	3,084	**3,192**	+108	+3.5%	25.5%
R&D Expenses	2,294	**3,175**	+881	+38.4%	24.4%
Operating Profit	2,066	**(296)**	(2,362)	(114.3%)	(5.5%)
Non-operating Income	520	**505**	(15)	(3.0%)	-
Non-operating Expenses	326	**466**	+139	+42.9%	-
Ordinary Profit	2,260	**(257)**	(2,518)	(111.4%)	(5.6%)
Extraordinary Income	-	**627**	+627	-	-
Extraordinary Losses	0	**0**	0	-	-
Profit before Income Taxes	2,260	**369**	(1,891)	(83.7%)	-
Income Taxes	650	**168**	(482)	(74.2%)	-
Profit Attributable to Owners of Parent	1,610	**201**	(1,408)	(87.5%)	5.4%
Reference: R&D Expenses before Deducting Contribution Amount by Collaborative R&D Destinations	2,469	**3,462**	+992	+40.2%	23.0%

Additional Remarks

- The decrease in Cost of Sales due to the decrease in Sales of Core Products.
- Non-operating Expenses include depreciation of Kobe Science Park Centre(KSPC). Non-current assets eligible for grants for KSPC are expected to be offset by special suspense account for tax purpose reduction entry by the end of the current financial year.
- Gain on cancellation of contract is accounted for Extraordinary Income.

(Unit: number of people)

	As of June 30,2023	As of June 30,2024	Difference
Number of Employees (Consolidated)	911	**991**	+80

Net Sales	FY2023 Q1	FY2024 Q1	Rate of change
Cost of Sales Ratio	31.1%	**25.5%**	(5.7%)
Cost of Sales Ratio *Excluding income from contractual payment	36.6%	**25.5%**	(11.1%)
R&D Expenses Ratio	21.2%	**39.0%**	+17.8%

YTD: year to date

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Breakdown of Net Sales (Consolidated)

(Unit: million yen)

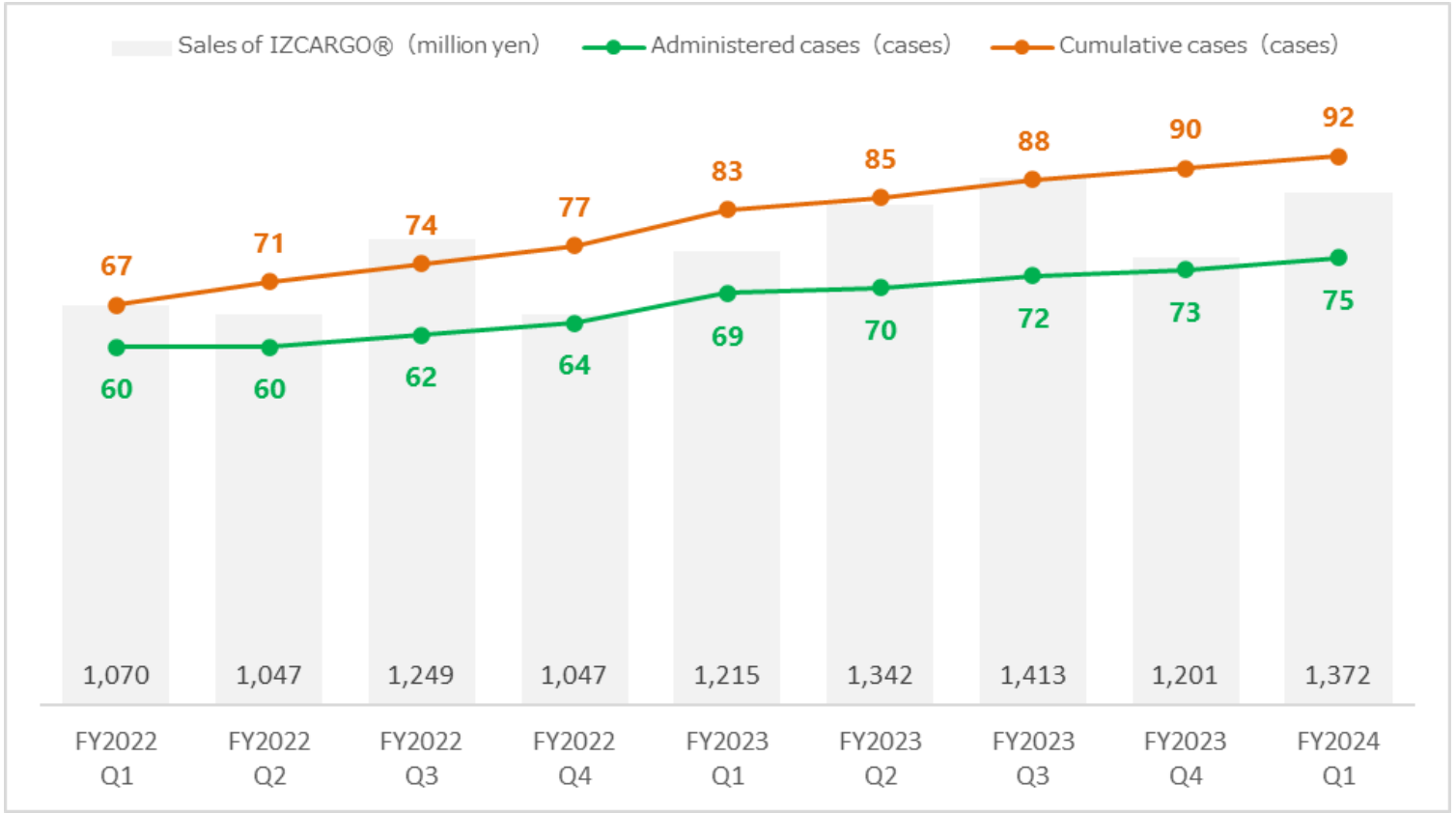
Consolidated	FY2023	FY2024			
	Q1 YTD	Q1 YTD	Year-on-year		Progress Rate
			Difference	Rate of change	
GROWJECT®	4,222	**4,649**	+426	+10.1%	25.4%
IZCARGO®	1,214	**1,372**	+158	+13.0%	24.1%
TEMCELL®HS Inj.	1,063	**730**	(333)	(31.4%)	26.1%
Treatments for renal anemia	1,615	**907**	(707)	(43.8%)	21.6%
Epoetin Alfa BS Inj. [JCR]	602	**515**	(87)	(14.5%)	23.4%
Darbepoetin Alfa BS Inj. [JCR]	1,012	**392**	(620)	(61.3%)	19.6%
Agalsidase Beta BS I.V. Infusion [JCR]	471	**277**	(194)	(41.2%)	25.2%
Total Core Products	8,587	**7,936**	(650)	(7.6%)	24.7%
Income from contractual payment	1,612	**15**	(1,596)	(99.0%)	0.2%
Other	609	**193**	(416)	(68.3%)	17.5%
Total Net Sales	10,808	**8,145**	(2,663)	(24.6%)	19.7%

Additional Remarks

- Sales of GROWJECT® and IZCARGO® increased by more than 10% year-on-year, progressing well.
- Sales of TEMCELL® were down 31.4% year-on-year, but sales progressed as planned against the full-year forecast. The full-year forecast is expected to be 13.5% lower than FY2023's results due to changes in the competitive environment.
- Sales of treatments for renal anemia were in line with the supply plan to our sales partner, Kissei Pharmaceutical Co., Ltd.
- Sales of Agalsidase Beta BS I.V. Infusion [JCR] were in line with the supply plan to our sales partner, Sumitomo Pharma Co., Ltd.
- Income from contractual payment is expected to be biased toward the second half of the year.

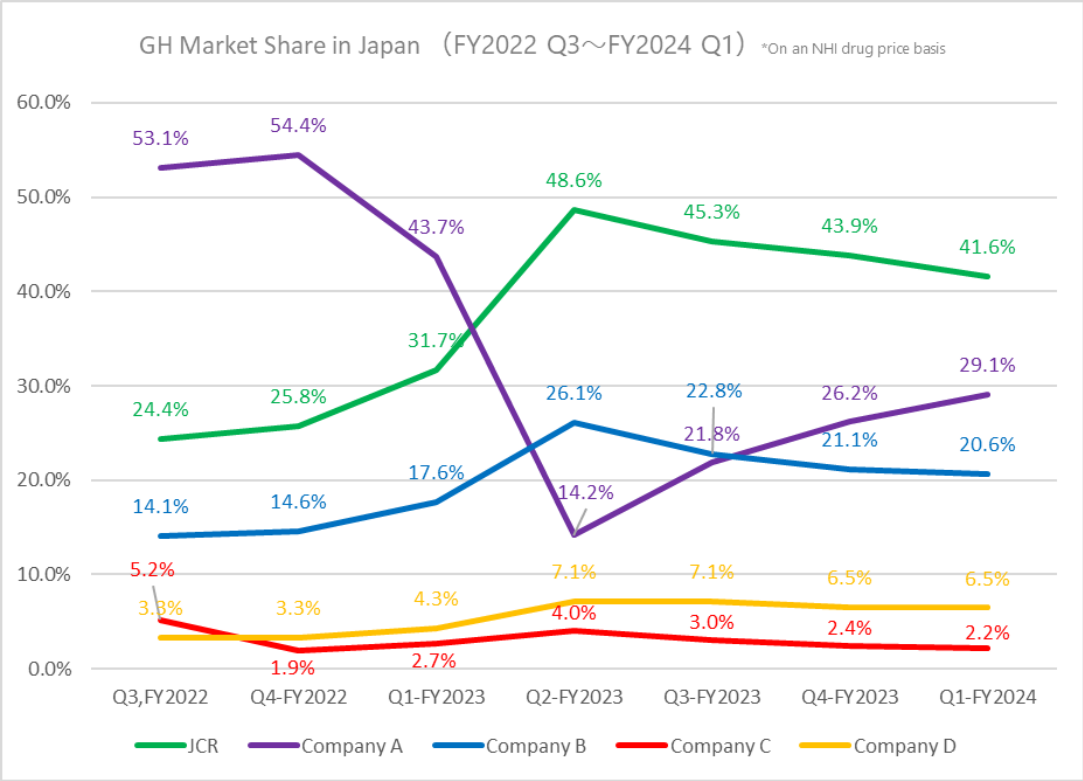
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IZCARGO® Prescription Status in Japan



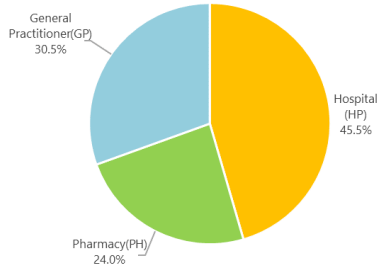
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GROWJECT® Market Share Trends in Japan (Quarterly)



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■ Human Growth Hormone Market in Japan



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■ GROWJECT® Market Share by buyer

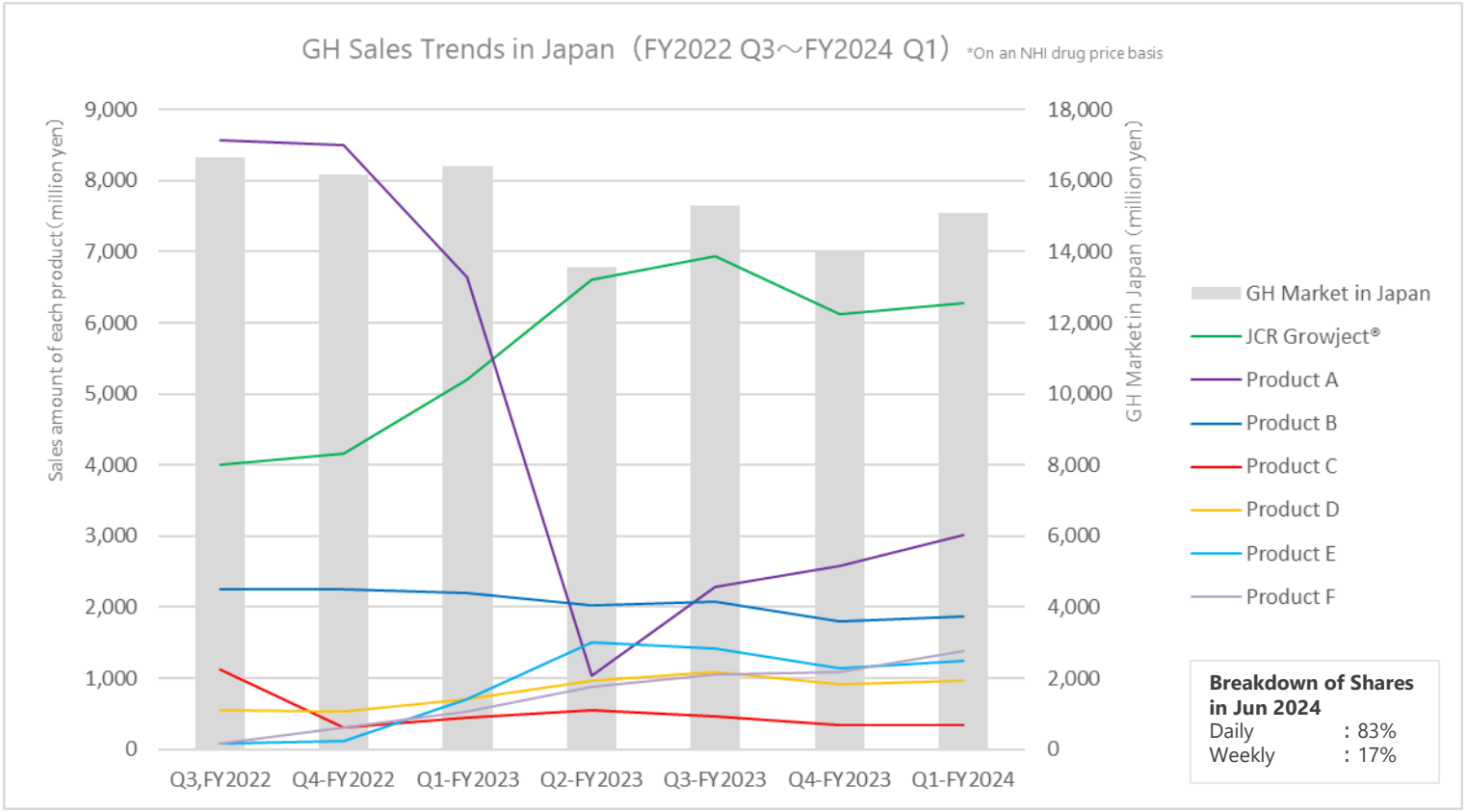
	Jun 2024	Sales Change FY2024 Q1 (vs. FY2023 Q1) *On an NHI drug price basis
HP Market	33.6%	+520 million yen
PH Market	31.3%	+273 million yen
GP Market	62.1%	+279 million yen

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Market definition by JCR Reprinted with permission

HP: Hospital
GP: General Practitioner
PH: Pharmacy

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GROWJECT® Sales Trends in Japan (Quarterly)



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FY2024 Progress of Developmental Pipelines

JR-141

BBB-penetrating iduronate-2-sulfatase (rDNA origin)
Indication: MPS type II
Brand name: IZCARGO® (Launched only in Japan, INN：pabinafusp alfa)

- Global Phase III study (JR-141-GS31: STARLIGHT study)
  - Completed enrollment of more than 60% of the eligible patients in Cohort A
- Revised package insert of IZCARGO® in Japan

JR-441

BBB-penetrating heparan N-sulfatase (rDNA origin)
Indication: MPS type IIIA

- Phase I/II study (JR-441-101)
  - Completed enrollment of the target number of 12 patients

JR-446

BBB-penetrating α-N-acetylglucosaminidase (rDNA origin)
Indication: MPS type IIIB

- Phase I/II study (JR-446-101)
  - Completed the clinical trial notification process in Japan

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FY2024 Progress of Developmental Pipelines

JR-142

Long-acting growth hormone (rDNA origin)
Indication: Pediatric growth hormone deficiency

- Phase III study (JR-142-301)
  - Completed the clinical trial notification process in Japan

JR-031HIE

human (allogeneic) bone marrow-derived mesenchymal stem cells
Indication: Neonatal Hypoxic Ischemic Encephalopathy
Brand name: TEMCELL®HS Inj.

- Decided to discontinue development based on the results of Phase I/II study

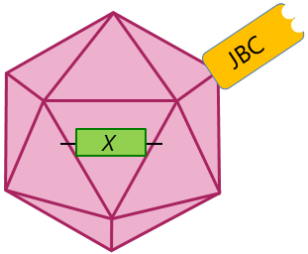
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R&D Pipeline

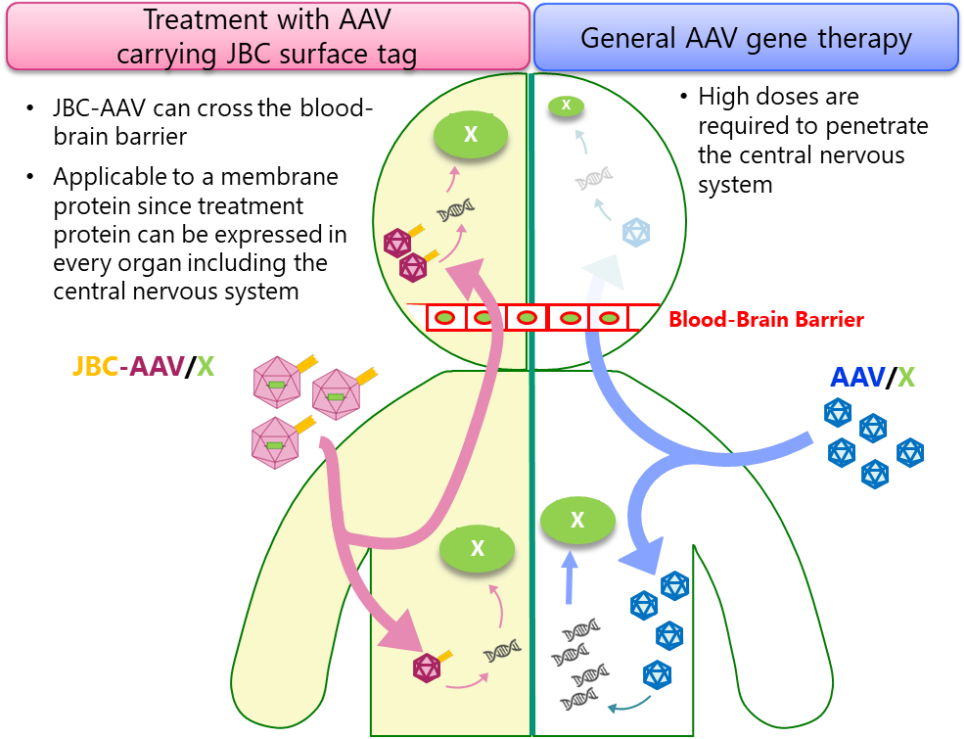
Code	Indication	Status	Upcoming Milestones
JR-141	MPS II (Hunter syndrome)	Global Ph3	• ~FY2027: Approval in US, EU, Brazil
JR-171	MPS I (Hurler syndrome etc.)	Global Ph1/2 completed	• Extension study is ongoing • Licensing out under negotiation
JR-142	Pediatric GHD	Ph2 (Analysis completed)	• FY2024: Phase 3
JR-441	MPS IIIA (Sanfilippo syndrome type A)	Global Ph1/2	• 2nd Half FY2025: 1-year clinical data is expected
JR-446	MPS IIIB (Sanfilippo syndrome type B)	CTN process completion	• 1st Half FY2024: FPI in Phase 1/2
JR-471	Fucosidosis	Pre-clinical	—

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- **Announced platform technology of gene therapy applied J-Brain Cargo®**
  - July The 7th International Forum of Lysosomal Disorders
  - October The 31st Annual Congress of the European Society of Gene & Cell Therapy (planned)



J-Brain Cargo®(JBC)-modified adeno-associated virus (JBC-AAV)



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Appendix

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Consolidated Financial Results - FY2024 Forecast

Reach Beyond, Together

(Unit: million yen)

Consolidated	FY2023	FY2024		
	Results	Forecast	Year-on-year	
			Difference	Rate of change
Net Sales	42,871	**41,300**	(1,571)	(3.7%)
Cost of Sales	11,620	**10,400**	(1,220)	(10.5%)
Gross Profit	31,251	**30,900**	(351)	(1.1%)
Selling, General and Administrative Expenses	23,719	**25,500**	+1,781	+7.5%
SG&A Expenses	12,484	**12,500**	+16	+ 0.1%
R&D Expenses	11,234	**13,000**	+1,766	+ 15.7%
Operating Profit	7,531	**5,400**	(2,131)	(28.3%)
Ordinary Profit	7,264	**4,600**	(2,664)	(36.7%)
Profit attributable to owners of parent	5,507	**3,700**	(1,807)	(32.8%)
Reference :R&D expenses before deducting contribution amount by collaborative R&D destinations	12,787	**15,062**	+2,275	+17.8%

Net sales	FY2023	FY2024 (Forecast)	YoY Rate of change
Cost of sales ratio	27.1%	**25.2%**	(1.9%)
Cost of sales ratio *Excluding Income from contractual payment	32.8%	**31.3%**	(1.4%)
R&D expense ratio	26.2%	**31.5%**	+5.3%
Operating profit ratio	17.6%	**13.1%**	(4.5%)

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Consolidated Breakdown of Net Sales – FY2024 Forecast

(Unit: million yen)

	FY2023	FY2024		
	Results	Forecast	Year-on-year	
			Difference	Rate of change
GROWJECT®	17,913	**18,300**	+387	+2.2%
IZCARGO®	5,171	**5,700**	+529	+10.2%
TEMCELL®HS Inj.	3,236	**2,800**	(436)	(13.5%)
Treatments for renal anemia	4,652	**4,200**	(452)	(9.7%)
Epoetin Alfa BS Inj. [JCR]	1,994	**2,200**	+206	+10.3%
Darbepoetin Alfa BS Inj. [JCR]	2,658	**2,000**	(658)	(24.8%)
Agalsidase Beta BS I.V. Infusion [JCR]	1,661	**1,100**	(561)	(33.8%)
Total Core Products	32,636	**32,100**	(536)	(1.6%)
Income from contractual payment	7,413	**8,100**	+687	+9.3%
Other	2,820	**1,100**	(1,720)	(61.0%)
Total Net Sales	42,871	**41,300**	(1,571)	(3.7%)

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Abbreviations （A～G）

AAV	Adeno-Associated Virus	アデノ随伴ウイルス
BBB	Blood-Brain Barrier	血液脳関門
CNS	Central Nervous System	中枢神経系
CSF	Cerebrospinal fluid	脳脊髄液
CTN	Clinical Trial Notification	治験計画届
EC	European Commission	欧州委員会
EMA	European Medicines Agency	欧州医薬品庁
ERT	Enzyme Replacement Therapy	酵素補充療法
EU	European Union	欧州連合
FDA	Food and Drug Administraion	米国食品医薬品局
GHD	Growth Hormone Deficiency	成長ホルモン分泌不全性低身長症

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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
i.v.	Intravenous Injection	静脈注射
JBC	J-Brain Cargo®	-
MPS	Mucopolysaccharidosis	ムコ多糖症
NPS	Named Patient Supply	特定の患者への医薬品提供プログラム
ODD	Orphan Drug Designation	希少疾病用医薬品指定
Ph I	Phase I	臨床第１相試験
Ph II	Phase II	臨床第２相試験
Ph III	Phase III	臨床第３相試験
PRIME	Priority Medicines	アンメットメディカルニーズを対象とした医薬品の開発支援を強化するためのスキーム
R&D	Research and Development	研究開発
TBD	To be determined	未定

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