



JCR Pharmaceuticals Co., Ltd.

Q3 Financial Results Briefing for the Fiscal Year Ending March 2025

January 31, 2025

Event Summary

[Company Name]	JCR Pharmaceuticals Co., Ltd.	
[Company ID]	4552-QCODE	
[Event Language]	JPN	
[Event Type]	Earnings Announcement	
[Event Name]	Q3 Financial Results Briefing for the Fiscal Year Ending March 2025	
[Fiscal Period]	FY2025 Q3	
[Date]	January 31, 2025	
[Number of Pages]	26	
[Time]	18:30 – 19:31 (Total: 61 minutes, Presentation: 25 minutes, Q&A: 36 minutes)	
[Venue]	Webcast	
[Venue Size]		
[Participants]		
[Number of Speakers]	3	
	Yoh Ito	Senior Executive Officer, Corporate Strategy, Executive Director, Corporate Strategy Division
	Naoki Kawata	Director, Corporate Strategy Department, Corporate Strategy Division
	Yoshihiro Oota	Director, Accounting Department, Corporate Strategy Division
[Analyst Names]*	Hidemaru Yamaguchi	Citigroup Securities
	Fumiyoshi Sakai	UBS Securities
	Kazuaki Hashiguchi	Daiwa Securities
	Shinichiro Muraoka	Morgan Stanley MUFG Securities
	Miyabi Yamakita	Jefferies Japan Ltd.
	Kota Maeda	Nomura Securities
	Hiroyuki Matsubara	Nomura Securities

Support

Japan 050.5212.7790
Tollfree 0120.966.744

North America 1.800.674.8375
Email Support support@scriptasia.com



Yo Mizuno

Tokio Marine Asset Management

*Analysts that SCRIPTS Asia was able to identify from the audio who spoke during Q&A or whose questions were read by moderator/company representatives.

Support

Japan 050.5212.7790
Tollfree 0120.966.744

North America 1.800.674.8375
Email Support support@scriptsasia.com



Presentation

Moderator: We will now hold a conference call to announce the financial results of JCR Pharmaceuticals Co., Ltd. for Q3 of the fiscal year ending March 31, 2025.

A script of today's presentation and Q&A session will be available on our website at a later date.

First, let me introduce today's speakers.

Yoh Ito, Senior Executive Officer, Executive Director, Corporate Strategy Division.

Ito: Hello.

Moderator: Naoki Kawata, Director, Corporate Strategy Department, Corporate Strategy Division.

Kawata: Hello.

Moderator: Lastly, Yoshihiro Ohta, Director, Accounting Department, Corporate Strategy Division.

Ohta: Hello.

Moderator: These are the three speakers for today.

The material to be used today has been posted on our website since 16:00 on January 31, 2025. If you need the material at hand, please visit that website.

Today's briefing will last approximately one hour, including the presentation and Q&A session. Questions will be taken in batches after all presentations have been completed. Approximately 40 minutes will be allotted for the Q&A session.

Mr. Ito will now explain the financial results for Q3 of the fiscal year ending March 31, 2025.

Support

Japan 050.5212.7790
Tollfree 0120.966.744

North America 1.800.674.8375
Email Support support@scriptsasia.com



1	FY2024 Third Quarter Consolidated Financial Results
2	Progress of Developmental Pipelines
3	Revision of FY2024 Consolidated Financial Forecast

Ito: I would like to explain the summary of the financial results for Q3 of the fiscal year ending March 31, 2025, which was disclosed today at 16:00.

Today, as you can see, I would like to explain the consolidated financial results announced today, the progress of developmental pipelines, and the revision of the consolidated financial forecast announced at the same time.

Overview: Consolidated Financial Results

Consolidated	FY2023		FY2024		Additional Remarks
	Q3 YTD	Q3 YTD	Year-on-year		
			Difference	Ratio	
Net Sales	33,718	25,880	(7,837)	(23.2%)	<ul style="list-style-type: none"> Cost to sales ratio excluding contractual payment remained favorable Depreciation from the new formulation facility at Kobe Science Park Center is included in General and Administrative Expenses and is expected to be offset against the "Special suspense account for tax purpose reduction entry" (by advanced depreciation) account by fiscal year-end The increase in R&D Expenses was mainly due to the establishment of overseas development structures and the advancement of clinical trials Extraordinary Income includes from Gain on reversal of share acquisition rights and Gain on cancellation of contract
Cost of Sales	8,423	7,007	(1,415)	(16.8%)	
Gross Profit	25,295	18,873	(6,422)	(25.4%)	
Selling, General and Administrative Expenses	17,486	19,627	+2,141	+12.2%	
SG&A Expenses	9,127	9,702	+575	+6.3%	
R&D Expenses	8,359	9,925	+1,566	+18.7%	
Operating Profit	7,809	(754)	(8,563)	-	
Non-operating Income	575	200	(374)	(65.1%)	
Non-operating Expenses	995	827	(168)	(16.9%)	
Ordinary Profit	7,388	(1,380)	(8,769)	-	
Extraordinary Income	0	1,065	+1,064	-	
Extraordinary Losses	18	2	(15)	(88.5%)	
Profit before Income Taxes	7,371	(317)	(7,688)	-	
Income Taxes	2,210	258	(1,951)	(88.3%)	
Profit Attributable to Owners of Parent	5,160	(576)	(5,737)	-	
Reference: R&D Expenses before Deducting Contribution Amount by Collaborative R&D Destinations	9,508	11,121	+1,613	+17.0%	

	FY2023 Q3 YTD	FY2024 Q3 YTD	Difference
Net Sales			
Cost of Sales Ratio	25.0%	27.1%	+2.1%
Cost of Sales Ratio *Excluding income from contractual payment	31.7%	27.6%	(4.0%)
R&D Expenses Ratio	24.8%	38.3%	+13.6%
Operating Profit Ratio	23.2%	(2.9%)	(26.1%)

YTD: year to date 3

Here is the summary of the consolidated financial results.

Support

Japan 050.5212.7790
Tollfree 0120.966.744

North America 1.800.674.8375
Email Support support@scriptasia.com

Net sales were JPY25,880 million, operating profit was negative JPY754 million, and profit attributable to owners of parent was negative JPY576 million. Compared to the previous year's nine-month period, sales and profits decreased.

Breakdown of Net Sales (Consolidated)



(Unit: million yen)

Consolidated	FY2023	FY2024		
	Q3 YTD	Q3 YTD	Year-on-year	
			Difference	Ratio
GROWJECT®	13,995	14,177	+182	+1.3%
IZCARGO®	3,969*	4,456	+486	+12.3%
TEMCELL®HS Inj.	2,699	2,296	(402)	(14.9%)
Treatments for renal anemia	3,673	2,595	(1,078)	(29.3%)
Epoetin Alfa BS Inj. [JCR]	1,681	1,250	(430)	(25.6%)
Darbepoetin Alfa BS Inj. [JCR]	1,992	1,345	(647)	(32.5%)
Agalsidase Beta BS I.V. Infusion [JCR]	998	1,149	+150	+15.1%
Total Core Products	25,336	24,675	(660)	(2.6%)
Income from contractual payment	7,112	517	(6,595)	(92.7%)
Other	1,269*	688	(581)	(45.8%)
Total Net Sales	33,718	25,880	(7,837)	(23.2%)

Additional Remarks

- Sales of GROWJECT® increased by 1.3% year-on-year, reflecting steady growth.
- Sales of IZCARGO® grew by 12.3% year-on-year, demonstrating strong performance
- Sales of TEMCELL®HS Inj. fell 14.9% year-on-year due to increased competition but remain steady against the full-year forecast.
- Epoetin Alfa BS Inj. [JCR] Expected to Fall Below Initial Plan
- Sales of Agalsidase Beta BS I.V. Infusion [JCR] are driven by strong performance at the distributor, Sumitomo Pharma Co., Ltd.
- Contract Revenue Includes Milestones from Joint Research; Major Revenue Expected in Q4
- The decline in other sales stems from lower contract manufacturing revenue.

*The sales of IZCARGO® under NPS program were removed and reclassified to "Other". As such, the respective figures of FY2023 Q3 shown here are different from those published on January 26, 2024.

YTD: year to date 4

Before going into the details of the financial results, I would like to explain the breakdown of net sales.

Overall, as I mentioned earlier, net sales totaled JPY25,880 million.

The sales of GROWJECT totaled JPY14,177 million in the cumulative Q3, an increase of JPY182 million over the same period last year. This is a solid trend, as it has been through Q2, and is on track against the plan.

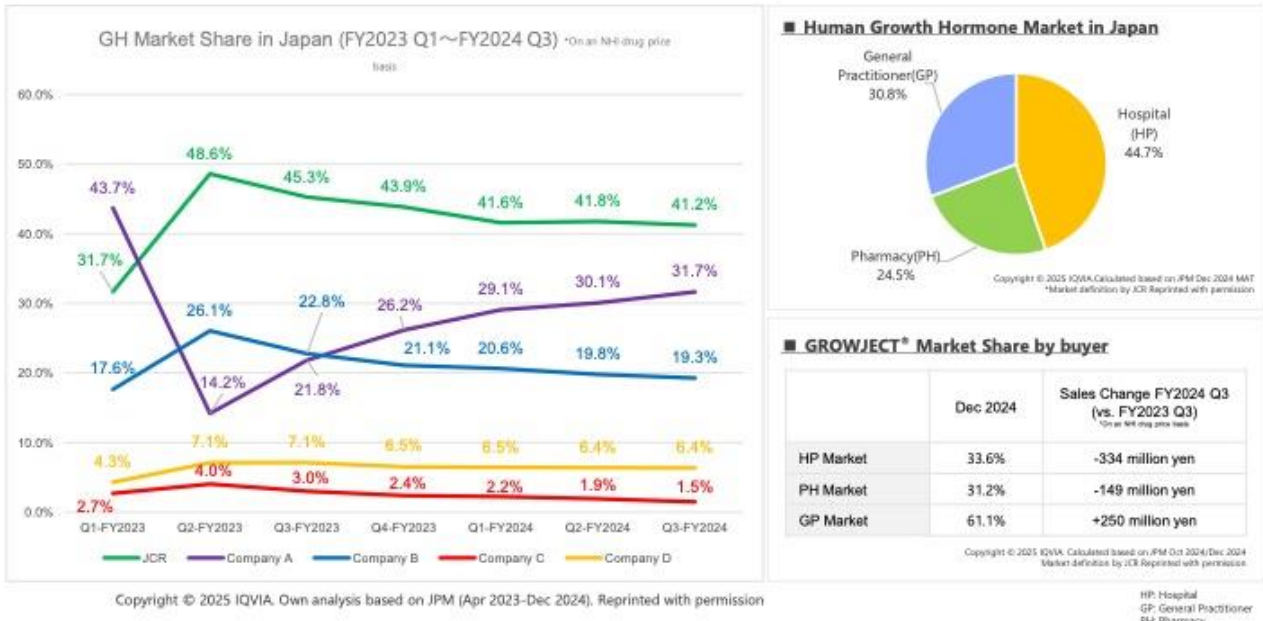
Support

Japan 050.5212.7790
Tollfree 0120.966.744

North America 1.800.674.8375
Email Support support@scriptasia.com



GROWJECT® Market Share Trends in Japan (Quarterly)



Let me explain a little more about GROWJECT. Please see page one of the appendix.

This is the trend of market share, which we always show you.

Our market share for Q3 is 41.2%, which is the figure on the far right of the green line. This has remained largely unchanged from 2Q.

Since market share is a percentage of the total market, it fluctuates depending on many factors. Looking at sales, I think you can see that sales for this Q3 alone were JPY4.776 billion, which is a steady increase compared to JPY4.752 billion in Q2 or JPY4.649 billion in Q1.

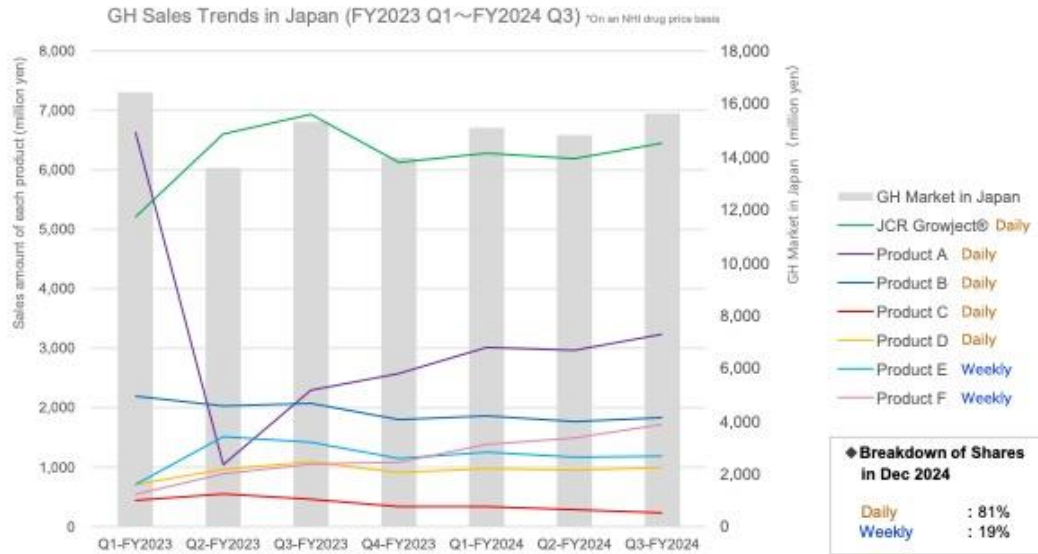
In addition, although the figures are not shown here, the capture rate of new patients is currently 48%, and is also steady. We believe this is the foundation from which we can further increase our market share.

Support

Japan 050.5212.7790
Tollfree 0120.966.744

North America 1.800.674.8375
Email Support support@scriptasia.com

GROWJECT® Sales Trends in Japan (Quarterly)



Copyright © 2025 IQVIA. Own analysis based on JPM (Apr 2023-Dec 2024). Reprinted with permission

12

What I would like you to see on the page I just showed you is breakdown of shares in the lower right-hand corner.

In December, the daily formulation had an 81% share and the weekly formulation had a 19% share. There has been no significant movement from the 82% share of the daily formulation and 18% share of the weekly formulation in September, and it remains the case that the weekly formulation is not growing rapidly.

Back to the sales breakdown. Next, let's look at IZCARGO.

Breakdown of Net Sales (Consolidated)

(Unit: million yen)

Consolidated	FY2023		FY2024	
	Q3 YTD	Q3 YTD	Year-on-year	
			Difference	Ratio
GROWJECT®	13,995	14,177	+182	+1.3%
IZCARGO®	3,969*	4,456	+486	+12.3%
TEMCELL®HS Inj.	2,699	2,296	(402)	(14.9%)
Treatments for renal anemia	3,673	2,595	(1,078)	(29.3%)
Epoetin Alfa BS Inj. [JCR]	1,681	1,250	(430)	(25.6%)
Darbepoetin Alfa BS Inj. [JCR]	1,992	1,345	(647)	(32.5%)
Agalsidase Beta BS I.V. Infusion [JCR]	998	1,149	+150	+15.1%
Total Core Products	25,336	24,675	(660)	(2.6%)
Income from contractual payment	7,112	517	(6,595)	(92.7%)
Other	1,269*	688	(581)	(45.8%)
Total Net Sales	33,718	25,880	(7,837)	(23.2%)

Additional Remarks

- Sales of GROWJECT® increased by 1.3% year-on-year, reflecting steady growth.
- Sales of IZCARGO® grew by 12.3% year-on-year, demonstrating strong performance
- Sales of TEMCELL®HS Inj. fell 14.9% year-on-year due to increased competition but remain steady against the full-year forecast.
- Epoetin Alfa BS Inj. [JCR] Expected to Fall Below Initial Plan
- Sales of Agalsidase Beta BS I.V. Infusion [JCR] are driven by strong performance at the distributor, Sumitomo Pharma Co., Ltd.
- Contract Revenue Includes Milestones from Joint Research; Major Revenue Expected in Q4
- The decline in other sales stems from lower contract manufacturing revenue.

*The sales of IZCARGO® under NPS program were removed and reclassified to "Other". As such, the respective figures of FY2023 Q3 shown here are different from those published on January 26, 2024.

YTD: year to date 4

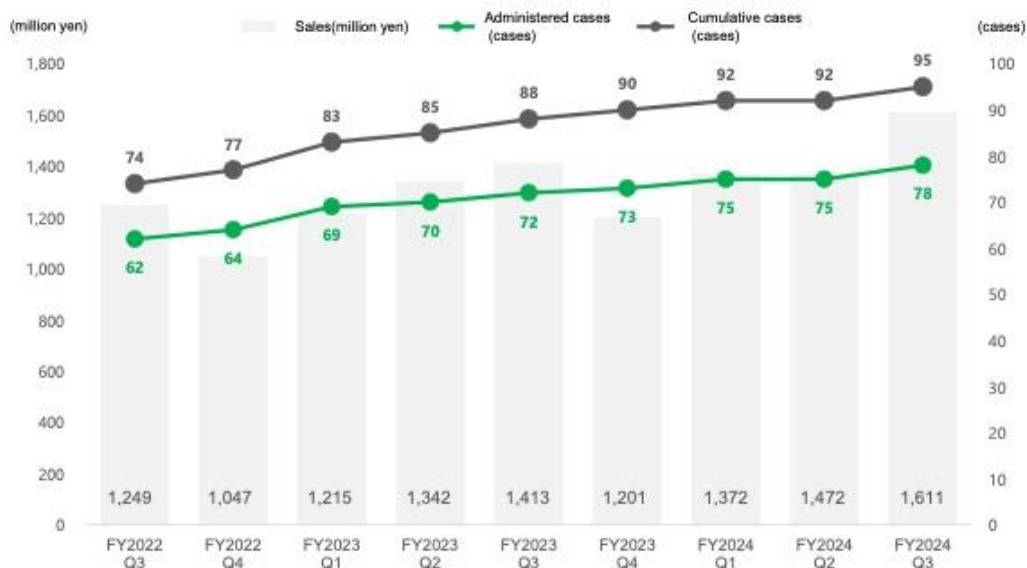
Support

Japan 050.5212.7790
Tollfree 0120.966.744

North America 1.800.674.8375
Email Support support@scriptasia.com

Cumulative sales of IZCARGO for Q3 totaled JPY4,456 million. Sales increased JPY486 million, or 12.3%, from the same period of the previous year, and were also favorable. This is also performing well against the plan.

IZCARGO® Prescription Status in Japan



13

This is the number of patients, which we always show you.

See the green line. As of the end of Q3, the number of cases administered was 78. So far this fiscal year, we have acquired five new cases.

As was the case through Q2, the absence of withdrawal cases is a key feature of this quarter.

In addition, the revision of the package insert we mentioned earlier and the shorter dosing times due to the elimination of the maximum dosage rate limit have contributed to switching from other drugs in some cases.

This includes examples of adults with time constraints due to work commitments who have switched due to shorter dosing time. I think we can also say that those are usually adults and heavier, so they tend to have a greater number of dosing vials.

As a result, IZCARGO is performing well.

Let's go back to the sales breakdown.

Support

Japan 050.5212.7790
Tollfree 0120.966.744

North America 1.800.674.8375
Email Support support@scriptsasia.com



Breakdown of Net Sales (Consolidated)

(Unit: million yen)

Consolidated	FY2023	FY2024		
	Q3 YTD	Q3 YTD	Year-on-year	
			Difference	Ratio
GROWJECT®	13,995	14,177	+182	+1.3%
IZCARGO®	3,969*	4,456	+486	+12.3%
TEMCELL®HS Inj.	2,699	2,296	(402)	(14.9%)
Treatments for renal anemia	3,673	2,595	(1,078)	(29.3%)
Epoetin Alfa BS Inj. [JCR]	1,681	1,250	(430)	(25.6%)
Darbepoetin Alfa BS Inj. [JCR]	1,992	1,345	(647)	(32.5%)
Agalsidase Beta BS I.V. Infusion [JCR]	998	1,149	+150	+15.1%
Total Core Products	25,336	24,675	(660)	(2.6%)
Income from contractual payment	7,112	517	(6,595)	(92.7%)
Other	1,269*	688	(581)	(45.8%)
Total Net Sales	33,718	25,880	(7,837)	(23.2%)

Additional Remarks

- Sales of GROWJECT® increased by 1.3% year-on-year, reflecting steady growth.
- Sales of IZCARGO® grew by 12.3% year-on-year, demonstrating strong performance
- Sales of TEMCELL®HS Inj. fell 14.9% year-on-year due to increased competition but remain steady against the full-year forecast.
- Epoetin Alfa BS Inj. [JCR] Expected to Fall Below Initial Plan
- Sales of Agalsidase Beta BS I.V. Infusion [JCR] are driven by strong performance at the distributor, Sumitomo Pharma Co., Ltd.
- Contract Revenue Includes Milestones from Joint Research; Major Revenue Expected in Q4
- The decline in other sales stems from lower contract manufacturing revenue.

*The sales of IZCARGO® under NPS program were removed and reclassified to "Other". As such, the respective figures of FY2023 Q3 shown here are different from those published on January 26, 2024.

YTD: year to date 4

TEMCELL sales were JPY2,296 million, down JPY400 million YoY. As announced, we originally set the sales target low for this fiscal year, and this is because of the competitive drug. The plan was set based on changes in the competitive environment. However, the result has exceeded the plan, and I think we can say that this is doing better than expected.

Of the renal anemia drugs below, sales of Epoetin Alfa are expected to fall short of the plan at the beginning of the fiscal year. I will explain this again in the section on consolidated forecast revisions.

Sales of Darbepoetin Alfa decreased by JPY647 million from the previous year to JPY1,345 million. This was a decrease from the prior year, but remained largely in line with our plan. As I will explain again later, we expect the full-year figure to be in line with our plan.

As you can see, Agalsidase Beta sold by Sumitomo Pharma Co., Ltd. increased by JPY150 million from the previous year and is performing well.

As a result, total sales of core products amounted to JPY24,675 million, down JPY660 million from the same period last year.

Income from contractual payment for the cumulative Q3 totaled JPY517 million, a decrease of JPY6.5 billion from the previous year. The figure through Q2 was JPY15 million. During Q3, as noted as an additional remark on the right, we achieved milestones in collaborative research with other companies and received a milestone payment. There are also milestone revenues related to the sale, which contributed to the JPY517 million in sales recorded. In addition, the Company expects to record major contract revenues in Q4.

Other sales totaled JPY688 million, down JPY581 million from the same period last year. This was due to a decrease in contract manufacturing revenue, as described on the right.

As a result, sales were JPY25.8 billion, a decrease of JPY7.8 billion from the previous year.

Support

Japan 050.5212.7790
Tollfree 0120.966.744

North America 1.800.674.8375
Email Support support@scriptasia.com

Overview: Consolidated Financial Results

Consolidated	(Unit : million yen)			
	FY2023	FY2024		
	Q3 YTD	Q3 YTD	Year-on-year	
			Difference	Ratio
Net Sales	33,718	25,880	(7,837)	(23.2%)
Cost of Sales	8,423	7,007	(1,415)	(16.8%)
Gross Profit	25,295	18,873	(6,422)	(25.4%)
Selling, General and Administrative Expenses	17,486	19,627	+2,141	+12.2%
SG&A Expenses	9,127	9,702	+575	+6.3%
R&D Expenses	8,359	9,925	+1,566	+18.7%
Operating Profit	7,809	(754)	(8,563)	-
Non-operating Income	575	200	(374)	(65.1%)
Non-operating Expenses	995	827	(168)	(16.9%)
Ordinary Profit	7,388	(1,380)	(8,769)	-
Extraordinary Income	0	1,065	+1,064	-
Extraordinary Losses	18	2	(15)	(88.5%)
Profit before Income Taxes	7,371	(317)	(7,688)	-
Income Taxes	2,210	258	(1,951)	(88.3%)
Profit Attributable to Owners of Parent	5,160	(576)	(5,737)	-
Reference: R&D Expenses before Deducting Contribution Amount by Collaborative R&D Destinations	9,508	11,121	+1,613	+17.0%

Net Sales	FY2023 Q3 YTD	FY2024 Q3 YTD	Difference
Cost of Sales Ratio	25.0%	27.1%	+2.1%
Cost of Sales Ratio "Excluding income from contractual payment"	31.7%	27.6%	(4.0%)
R&D Expenses Ratio	24.8%	38.3%	+13.6%
Operating Profit Ratio	23.2%	(2.9%)	(26.1%)

- Additional Remarks**
- Cost to sales ratio excluding contractual payment remained favorable
 - Depreciation from the new formulation facility at Kobe Science Park Center is included in General and Administrative Expenses and is expected to be offset against the "Special suspense account for tax purpose reduction entry" (by advanced depreciation) account by fiscal year-end
 - The increase in R&D Expenses was mainly due to the establishment of overseas development structures and the advancement of clinical trials
 - Extraordinary Income includes from Gain on reversal of share acquisition rights and Gain on cancellation of contract

YTD: year to date 3

Back to the overview of the financial results. Cost of sales was JPY7,007 million on sales of JPY25,800 million.

See the second column of the table below right, cost of sales ratio (excluding income from contractual payment).

The cost of sales ratio (excluding income from contractual payment) was 27.6% for the first nine months of this fiscal year, a 4% decrease from the previous year. As was the case through Q2, lot outs were low and the cost ratio itself fell due to on-site efforts. In addition, as I have just shown in the breakdown of sales, sales of products with low-cost ratios grew and sales of products with high-cost ratios did not. As a result, the product mix turned around in terms of cost ratio and gross profit, resulting in a lower cost ratio.

As a result, gross profit, which is net sales minus cost of sales, was JPY18.8 billion.

On the other hand, selling, general and administrative expenses totaled JPY19.6 billion.

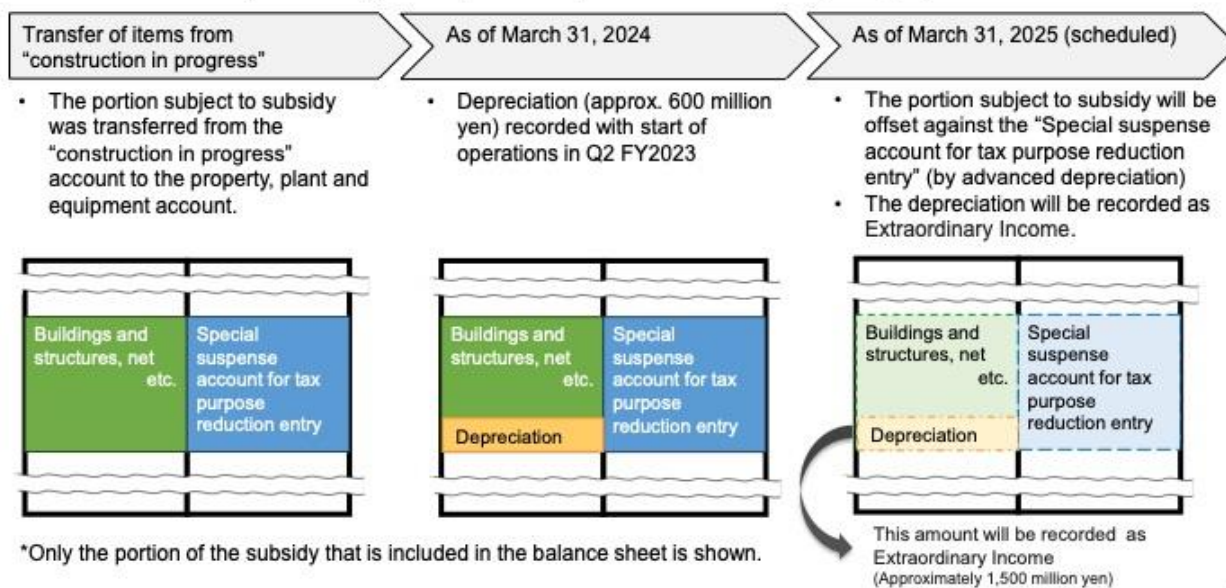
Selling, general and administrative expenses, excluding R&D expenses, were JPY9.7 billion, an increase of JPY575 million from the same period last year. This includes approximately JPY500 million in depreciation related to the construction of the API plant at the Kobe Science Park Center. As we mentioned previously, this is expected to be offset by the "special suspense account for tax purpose reduction entry" (by advanced depreciation) account by the end of the current fiscal year.

Support

Japan 050.5212.7790
Tollfree 0120.966.744

North America 1.800.674.8375
Email Support support@scriptasia.com

- In the third quarter financial statement, approximately 500 million yen of the depreciation at Kobe Science Park Center was recorded as SG&A expenses and approximately 100 million yen was recorded as Non-operating expenses.



The last page of the appendix includes the "Handling of subsidies" as a reference.

As you can see at the top, in Q3, we have included approximately JPY500 million of depreciation expense for the Kobe Science Park Center in SG&A expenses and approximately JPY100 million in non-operating expenses.

As noted on the far-left side of this slide, construction in progress was transferred to property, plant, and equipment when this asset became operational. As stated in the middle, as of the end of March 2024, it was already partially in operation and JPY600 million was recorded as depreciation expense.

It is assumed that the subsidy will be finalized as of the end of March 2025, at which point they will be offset against the special suspense account for tax purpose reduction entry on the liability side. By this so-called advanced depreciation, the depreciation estimated at approximately JPY1.5 billion accumulated up to this point will be recorded as extraordinary income.

As a result, an extraordinary income of JPY1.5 billion will be recorded in the current fiscal year, and approximately JPY800 million, including SG&A and non-operating expenses, is expected to be recorded as depreciation. As a result, we expect to end up with an overall profit of rounded JPY600 million. We will explain this again at the time of the year-end closing.

Let's return to the financial summary. R&D expenses totaled JPY9,925 million, up JPY1.5 billion from the same period last year. As we have mentioned in the past, this is due to the establishment of overseas development structures and advancement in clinical trials.

As a result, operating profit was negative JPY754 million. Operating profit through Q2 was negative JPY739 million, so it can be said that it remained almost flat in Q3.

As shown below that, JPY200 million of non-operating income and JPY827 million of non-operating expenses were recorded. Non-operating expenses include JPY433 million in equity in losses of affiliates. In addition, there is the depreciation expense of JPY140 million and the foreign exchange loss of JPY77 million that I mentioned earlier.

Support

Japan	050.5212.7790	North America	1.800.674.8375
Tollfree	0120.966.744	Email Support	support@scriptasia.com

Regarding equity in losses of affiliates, we did not record any equity in losses of affiliates in Q3 because we sold a portion of our holdings in Mycenax, a Taiwanese CDMO, and this company is no longer an affiliated company accounted for by the equity method, as we mentioned in our previous earnings announcement.

As a result, ordinary profit was negative JPY1,380 million and extraordinary income was JPY1,065 million, the latter including gain on reversal of share acquisition rights and gain on cancellation of contract already posted by Q2.

Profit before income taxes was negative JPY317 million and profit attributable to owners of parent was negative JPY576 million.

Progress of Developmental Pipelines



“First dosing in first subject” was achieved in 3 studies in 3 products

JR-441	BBB-penetrating heparan N-sulfatase (rDNA origin) Indication: MPS type IIIA	<ul style="list-style-type: none">- Japan: Phase I study (JR-441-JP11)
JR-446	BBB-penetrating α -N-acetylglucosaminidase (rDNA origin) Indication: MPS type IIIB	<ul style="list-style-type: none">- Japan: Phase I/II study (JR-446-101)
JR-142	Long-acting growth hormone (rDNA origin) Indication: Pediatric growth hormone deficiency	<ul style="list-style-type: none">- Japan: Phase III study (JR-142-301)

5

Next, I will explain the progress of the developmental pipelines.

The topic of Q3 is that the first dosing in first subject was achieved in three studies in three products, as described.

The first dose of JR-441, at the top, for the indication of mucopolysaccharidosis type IIIA, has started on October 31 in a Phase I study in Japan. It also received orphan designation in Japan on December 25.

The first dose of JR-446 has started on December 3 in a Phase I/II study in Japan.

JR-142 is a weekly formulation of growth hormone. The first dose has also started on December 19 in a Phase III study in Japan.

Support

Japan 050.5212.7790
Tollfree 0120.966.744

North America 1.800.674.8375
Email Support support@scriptasia.com



Overview of Clinical or late Preclinical Pipeline

Code	Indication	Status				Milestones/Comments
		Preclinical	Phase 1	Phase 2	Phase 3	
JR-141	MPS II (Hunter syndrome)	Global Ph3				<ul style="list-style-type: none"> Q3 FY2025: Enrollment completion ~FY2027: Approval in US, EU, Brazil
JR-171	MPS I (Hurler syndrome etc.)	Global Ph1/2 completed				<ul style="list-style-type: none"> Extension study ongoing Partnering intensified
JR-142	Pediatric GHD	Ph3 (Japan)				<ul style="list-style-type: none"> Dec 2024: Initiation of first dosing in Ph3
JR-441	MPS IIIA (Sanfilippo syndrome type A)	Ph1/2 (Germany)				<Ph1/2> <ul style="list-style-type: none"> Patient enrollment completed 2H FY2025: 1-year clinical data <Ph1> <ul style="list-style-type: none"> Oct 2024: Initiation of first dosing
		Ph1 (Japan)				
JR-446	MPS IIIB (Sanfilippo syndrome type B)	Ph1/2 (Japan)				<ul style="list-style-type: none"> Dec 2024: Initiation of first dosing in Ph1/2
JR-471	Fucosidosis					—

6

This is the status of our developmental pipeline, which we always show you.

We are still working on Global Phase III for JR-141 at the top of the list, and are in the process of registering cases. The approval schedule is for FY2027 and is unchanged from the previous description. We are assuming that an interim analysis of 60% of the subjects will be performed and submitted for application. However, we are in constant communication with the regulatory authorities, and we are always considering and making efforts to accelerate the approval period as much as possible.

Recruitment is on track and is expected to be 100% complete in Q3 of FY2025.

Negotiations are underway for out-licensing of JR-171. This will be discussed somewhat later.

As I mentioned earlier, the first dose of JR-142 in Phase III has been completed.

As I mentioned earlier, the first dose of JR-441 has also been completed in Phase I in Japan. In Phase I/II in Germany, we have completed the enrollment of the target number of patients and the clinical trial is underway, and the data analysis is scheduled to be completed in the second half of FY2025.

The first dose of JR-446 was completed in Phase I/II in Japan in December.

Support

Japan 050.5212.7790
Tollfree 0120.966.744

North America 1.800.674.8375
Email Support support@scriptasia.com

Revision of FY2024 Consolidated Financial Forecast

	Net Sales (Unit: million yen)	Operating Profit (Unit: million yen)	Ordinary Profit (Unit: million yen)	Profit Attributable to Owners of Parent (Unit: million yen)	Earnings Per Share (Unit: yen)
Previously announced forecasts	41,300	5,400	4,600	3,700	29.65
Revised forecasts	39,000	1,400	750	2,200	17.77
Difference	(2,300)	(4,000)	(3,850)	(1,500)	-
Ratio	(5.6%)	(74.1%)	(83.7%)	(40.5%)	-
Reference: FY2023 Results	42,871	7,531	7,264	5,507	44.13

Net Sales

- While product sales remain largely on track, income from contractual payment was revised downward because an overseas licensing agreement will not be concluded for JR-171 within this fiscal year.

Operating Profit

- Operating profit was revised downward to 1,400 million yen due to a decrease in gross profit resulting from lower net sales, coupled with increases in cost of sales and selling, general and administrative expenses.
 - ✓ Cost of Sales: Despite improvements in cost efficiency and a favorable product mix, higher disposal costs for manufacturing materials are expected. Accordingly, the cost of sales have increased by 700 million yen.
 - ✓ Expenses were increased by 400 million yen allocated to selling, general and administrative expenses, and 600 million yen to R&D expenses, based on results through the third quarter, etc.

7

Here you will find the revised consolidated earnings forecast announced today.

The full-year sales forecast was revised downward by JPY2.3 billion from the initial JPY41.3 billion to JPY39 billion.

As noted below, although product sales are generally in line with our plan, we have revised our forecast for income from contractual payment on the assumption that the overseas licensing agreement for JR-171 is not expected to be concluded during the current fiscal year.

See the next slide for a breakdown of sales.

Support

Japan 050.5212.7790
Tollfree 0120.966.744

North America 1.800.674.8375
Email Support support@scriptsasias.com

Revision of FY2024 Consolidated Financial Forecast

	Previously announced forecasts	Revised forecasts	Difference	Ratio	(Unit: million yen) Reference: FY2023 Results
GROWJECT®	18,300	18,300	-	-	17,913
IZCARGO®	5,700	5,800	+100	+1.8%	5,171
TEMCELL®HS Inj.	2,800	2,900	+100	+3.6%	3,236
Treatments for renal anemia	4,200	3,700	(500)	(11.9%)	4,652
Epoetin Alfa BS Inj. [JCR]	2,200	1,700	(500)	(22.7%)	1,994
Darbepoetin Alfa BS Inj. [JCR]	2,000	2,000	-	-	2,658
Agalsidase Beta BS I.V. Infusion [JCR]	1,100	1,100	-	-	1,661
Total Core Products	32,100	31,800	(300)	(0.9%)	32,636
Income from contractual payment	8,100	6,100	(2,000)	(24.7%)	7,413
Other	1,100	1,100	-	-	2,820
Total Net Sales	41,300	39,000	(2,300)	(5.6%)	42,871

8

First of all, no revision has been made to the already announced forecast for GROWJECT.

For IZCARGO, we have increased our sales forecast by JPY100 million to JPY5.8 billion, taking into account the current situation.

The sales forecast for TEMCELL was also revised upward by JPY0.1 billion to JPY2.9 billion.

Next, below that, the sales forecast for Epoetin Alfa, a treatment for renal anemia, was revised downward by JPY500 million to JPY1.7 billion.

Epoetin Alfa was recalculated as an unprofitable product in the 2024 NHI price revision. This increased the NHI price for the two standards. Revision rates for the other two standards were also relatively low. This caused switching to other drugs.

Also, sales are outsourced to Kissei Pharmaceutical Co., Ltd., and I believe Kissei has a policy of selling at higher prices. Fear of overstocking has led to fewer orders from Kissei. As a result, we have revised the forecast.

On the other hand, the forecast for Darbepoetin Alfa has not been revised from the initial forecast.

The same is true for Agalsidase Beta.

The total sales forecast for core products is JPY31.8 billion, a downward revision of JPY0.3 billion.

As I mentioned earlier, we expect income from contractual payment to decrease by JPY2 billion to JPY6.1 billion, based on the assumption that the license agreement for JR-171 is not expected this fiscal year.

The forecast for other remains unchanged. The forecast for total net sales is JPY39 billion, a downward revision of JPY2.3 billion.

Support

Japan 050.5212.7790
Tollfree 0120.966.744

North America 1.800.674.8375
Email Support support@scriptasia.com

Revision of FY2024 Consolidated Financial Forecast

	Net Sales (Unit: million yen)	Operating Profit (Unit: million yen)	Ordinary Profit (Unit: million yen)	Profit Attributable to Owners of Parent (Unit: million yen)	Earnings Per Share (Unit: yen)
Previously announced forecasts	41,300	5,400	4,600	3,700	29.65
Revised forecasts	39,000	1,400	750	2,200	17.77
Difference	(2,300)	(4,000)	(3,850)	(1,500)	-
Ratio	(5.6%)	(74.1%)	(83.7%)	(40.5%)	-
Reference: FY2023 Results	42,871	7,531	7,264	5,507	44.13

Net Sales

- While product sales remain largely on track, income from contractual payment was revised downward because an overseas licensing agreement will not be concluded for JR-171 within this fiscal year.

Operating Profit

- Operating profit was revised downward to 1,400 million yen due to a decrease in gross profit resulting from lower net sales, coupled with increases in cost of sales and selling, general and administrative expenses.
 - Cost of Sales: Despite improvements in cost efficiency and a favorable product mix, higher disposal costs for manufacturing materials are expected. Accordingly, the cost of sales have increased by 700 million yen.
 - Expenses were increased by 400 million yen allocated to selling, general and administrative expenses, and 600 million yen to R&D expenses, based on results through the third quarter, etc.

7

Back to the previous slide. Operating profit is expected to be JPY1.4 billion, a decrease of JPY4 billion from the initial forecast of JPY5.4 billion. One major cause is, as I mentioned earlier, the decline in gross profit due to lower sales. Since this is a decrease in license income, it directly translates into a decrease in gross profit.

As for other points, as I mentioned earlier, the cost of sales ratio through Q3 has declined 4% from last year, and we expect it to remain strong as it is. Gross profit is expected to increase due to an overall reduction in the cost ratio throughout the year.

On the other hand, disposal costs for manufacturing materials and other items are expected to increase. This is due to the expiration of materials procured in large quantities for the continuous stable supply during the COVID-19 pandemic. The cost of sales has been revised upward by JPY0.7 billion to reflect the expected increase in disposal costs.

In addition, selling, general and administrative expenses were revised upward by JPY0.4 billion. As I mentioned earlier, JPY600 million of this amount is depreciation expense for the Kobe Science Park Center, which will be offset by extraordinary income at the end of March.

Regarding R&D expenses, the progress of clinical trials overseas, including JR-141, has been more advanced than expected, and we have revised the amount of R&D expenses upward by JPY600 million due to these factors. As a result, operating profit has been revised to JPY1.4 billion.

The forecast for ordinary profit and profit attributable to owners of parent have been revised downward to JPY750 million and JPY2.2 billion, respectively.

That is all I have to say. Thank you very much.

Moderator: Thank you for your attention.

Support

Japan	050.5212.7790	North America	1.800.674.8375
Tollfree	0120.966.744	Email Support	support@scriptasia.com

Question & Answer

Moderator [M]: We will now move to the question-and-answer session.

Each person is limited to two questions at a time, and each question will be answered each time. You may raise your hand as many times as you like.

First, Mr. Yamaguchi, could you ask a question?

Yamaguchi [Q]: Thank you.

The first question is about the fact that you expect the JR-171 license agreement will not be concluded by the end of this fiscal year. I am sure that you have done a lot of things, and I think it is difficult in some aspects because there is a partner who you are dealing with. Is that just moving from this quarter to next, or is it becoming more difficult to enter into a licensing agreement in the first place?

Ito [A]: Thank you for your question.

Regarding JR-171, we had expected to sign a contract with a certain company in the current fiscal year, but due to a major move by that company, something like a structural reform, we were unable to conclude the contract. Therefore, we have determined that there will not be the revenue originally anticipated in this fiscal year.

Negotiations with other companies are ongoing at this time, and as we mentioned, negotiations for the out-licensing of JR-141 are also underway at the same time. Our intention is to license out them together, if possible. Some companies have also expressed interest in doing so, and we are now trying to negotiate with them.

Yamaguchi [Q]: I understand.

Second, as for JR-141 development, etc., in the context of competition, I think the timing of applications in the US is a bit accelerated for other companies. From your company, we have repeatedly heard whether or not you would like to apply for it with the data you have so far. Is there any possibility of an earlier filing date for JR-141 in the US?

Kawata [A]: I, Kawata of the Corporate Strategy Department, will answer that question.

We are currently in ongoing communication regarding negotiations with the FDA. We are not yet able to give specifics on the timing of the consultation, but we believe it will be during H1 of 2025. Interaction continues toward this end.

We are unable to answer this question at this time, as it depends on future consultation with the authorities as to what data will be used in the application.

That is all for our response at this stage.

Yamaguchi [Q]: I understand.

You mentioned a consultation in H1. Are you thinking that a successful meeting might lead to an application?

Support

Japan 050.5212.7790
Tollfree 0120.966.744

North America 1.800.674.8375
Email Support support@scriptasia.com



Kawata [A]: At this stage, as mentioned in the pipeline section, we are considering applying at the appropriate time with the aim of obtaining approval during the 2027 fiscal year. However, we will continue to negotiate to make that happen as soon as possible.

Yamaguchi [M]: I understand. Thank you very much. That is all.

Moderator [M]: Thank you very much.

Mr. Sakai, could you ask a question?

Sakai [Q]: My name is Sakai from UBS.

First of all, I would like to confirm, did you just say that you would like to license out JR-171 and JR-141 at the same time?

Ito [A]: Yes, these drugs are of a similar area, so we think it would be possible to license out them at the same time.

In fact, some companies have expressed interest in licensing-out of both drugs at the same time. Including those companies, we continue to talk with companies that have expressed interest in licensing out JR-141 or JR-171.

Sakai [Q]: Do you mean that JR-141 and JR-171 will be out-licensed to the same company at the same time?

Ito [A]: That is a possibility we are pursuing.

Sakai [Q]: That is a possibility, but you are pursuing it as I thought. Is that a relatively high priority? Is that more efficient for your company, since only one process will be needed?

Ito [A]: In that aspect, yes. We believe that it would be desirable and preferable for us to be able to license out the two together, depending on conditions.

Sakai [Q]: I understand. I would like to ask you two questions.

One is about TEMCELL. It is making progress against your plan as expected, and you have revised your forecast slightly upward this time. However, you mentioned that there is a downward trend, partly due to the influence of JAKAVI. Hasn't the downward trend been halted or is it being halted to some extent?

Kawata [A]: I would not say that the impact of JAKAVI was minor, but it was less than we had assumed at the time of planning at the beginning of the period.

This is due to the characteristics of TEMCELL. We believe that due to its characteristics of being easier to use than immunosuppressive agents, its position is probably well established in clinical practice.

Given this and the fact that JAKAVI is also gaining a certain degree of penetration, we expect that TEMCELL will be able to maintain its current level of sales in the future.

Sakai [Q]: I understand.

The significant impact on your company's bottom line of not being able to finalize the out-licensing agreement is clear from the downward revision. On the other hand, you have increased your forecast for SG&A and R&D expenses this time. If this situation continues, and if contracts are not finalized for the next fiscal year, a further decline in profits will be inevitable. I know that R&D is the initiative in your company, but to what

Support

Japan 050.5212.7790
Tollfree 0120.966.744

North America 1.800.674.8375
Email Support support@scriptasia.com



extent is this situation acceptable? As to whether it is acceptable, I think the definition of acceptable on our side and your company's side is a bit different. Can you give us some information of what kind of discussions you are having on your side?

Ito [A]: Thank you for your question.

As you have pointed out, our current profit/loss structure is such that gross profit from products alone is not sufficient to cover R&D expenses plus selling, general and administrative expenses.

There are some technical factors regarding the revised SG&A expenses. With regard to SG&A expenses, the actual expenses used in the business have remained below the plan, and we intend to keep this under control in the future.

As for R&D expenses, the structure of costs or revenues, as I mentioned earlier, is largely due to the significant increase in R&D expenses compared to the previous period and the period prior to that. So we think it is very important to control this.

We are in the process of drafting a budget for the next fiscal year. It is easy to infer, of course, that as these expenses continue to increase, it will be difficult to record a profit. Therefore, we would like to keep this under control.

In this context, it is important to know how to prioritize in research or development. At the same time, we believe that how to proceed with licensing negotiations will also be a very significant factor.

Furthermore, in the medium-term, or rather mid- to long-term, how do we maintain and improve product sales? Sales of IZCARGO are expected to continue to improve. On the other hand, for GROWJECT, we need to consider how to keep the NHI price revision rate low and how to increase sales by expanding indication the declining birthrate, and we are discussing these issues internally.

Sakai [Q]: I just have one more question. I believe Chairman Ashida mentioned something about not capping R&D expenses, has this idea changed yet?

Ito [A]: In our mid-term management plan, we have stated that we will invest in research and development expenses, and we naturally hold that view.

However, I think we need to think carefully about this, as well as whether or not we should continue to increase those expenses, as you have pointed out.

Sakai [M]: Thank you very much.

Moderator [M]: Thank you very much.

Mr. Hashiguchi, could you ask a question?

Hashiguchi [Q]: I am Hashiguchi from Daiwa Securities. Thank you.

First, how do you feel the external evaluation of the value of JR-171 is changing? From your earlier explanation, I understood that a contract had almost been finalized with a certain company, but is now being renegotiated from scratch. From your company's point of view, it sounds as if you are making concessions from the first candidate to the second and third candidates.

In addition, over time, the remaining term of JR-171 patents will shorten while competitors' development will advance. In general, I believe that the value of the product often decreases over time. I would like to know

Support

Japan 050.5212.7790
Tollfree 0120.966.744

North America 1.800.674.8375
Email Support support@scriptasia.com



how you feel that the changing environment regarding this drug has changed inquiries from outside and the level of interest from interested companies.

Kawata [A]: Thank you very much. I, Kawata, will respond.

As for the value of JR-171 itself, I think you are right that from a general perspective, it will decline over time. However, we understand that no leading competitive products have emerged. We believe that we are still in a situation where there is no treatment for patients who present with CNS symptoms that cannot be approached with existing drugs. In this sense, we believe that the role or value of our drugs has not changed significantly.

On the other hand, we understand that complex diseases such as Hurler, Hurler-Scheie or Scheie syndrome are difficult for various companies to evaluate. We are learning that.

Therefore, we will continue to negotiate with them by presenting the data we have and the non-clinical data of the drug.

As I mentioned earlier, we are now in ongoing discussions with companies that could understand such matters in our ongoing negotiations.

Hashiguchi [Q]: Thank you very much.

My second question is about your forecast for GROWJECT. You have not changed your forecast this time. What are your thoughts on the certainty of the achievement? If I am not mistaken, sales in the October-December period were down about 9% from the previous year, and to achieve this plan, I think sales will have to increase about 5% from the previous year in the January-March period. On a quarterly basis, of course, there will be fluctuations due to various factors. Could you please explain a little more about the situation?

Ito [A]: Thank you.

We have had various discussions with the sales division about GROWJECT, and they seem to be feeling quite good responses.

Many accounts switched to our products when Novo Nordisk could not supply their drugs. The decrease due to its reversion was much less than expected.

There are many factors, but one of them, as I have repeatedly mentioned, is the unique characteristics of our devices.

As I mentioned earlier, we believe that we can achieve this figure with a great degree of certainty, since we are acquiring more new patients than our market share.

Hashiguchi [M]: Thank you very much.

Moderator [M]: Thank you very much.

Mr. Muraoka, could you ask a question?

Muraoka [Q]: Thank you very much. I am Muraoka from Morgan Stanley.

Let me ask you about the JR-171 contractual payment, too. Considering that something that was pretty close to being concluded has been put off, and you will now start over, can you say that partnering can be expected in the next fiscal year, three months from now? Or should we assume that it will be after the next fiscal year?

Support

Japan 050.5212.7790
Tollfree 0120.966.744

North America 1.800.674.8375
Email Support support@scriptasia.com



And even if it were the next fiscal year, I felt that you could say that it would be just before the end of the fiscal year, just as it was this fiscal year, and we might have to repeat the thrill and the fear again in the next fiscal year. How should I interpret this, will it not take too long or do you have to take your time to decide?

Ito [A]: This is a difficult question to answer. I would like to refrain from saying at this time what speed we will proceed with.

We will now put together the budget for the next fiscal year. When we explain the plan for the next fiscal year, we will also explain the situation and how we are thinking about it.

Muraoka [Q]: Thank you very much.

You mentioned earlier that you have requested a meeting with the FDA regarding JR-141. By first half, do you mean the first half of the calendar year through June, or that of the fiscal year through September?

Kawata [A]: It mean the calendar year.

Muraoka [Q]: So you will have a meeting with the FDA by June, and you may get a good sign.

Kawata [A]: I can't answer whether we will get a good sign or not, but we are naturally expecting it.

Muraoka [Q]: That's just it. This is almost hypothetical, but the US administration has changed and the head of the FDA is also changing, although I don't mean RFK Jr. Do you feel that this could have an impact down the road and change the review policy, for example, if the person in charge of CBER changes and says, bring me endpoints, not biomarkers? Or, is it likely that this is not the case and that the direction of quick application with biomarker will remain the same?

Ito [A]: I can only answer that it is unknown at this stage.

Muraoka [Q]: Is there anything you can say, even at the rumor level?

Kawata [A]: Not at the rumor level, but based on published articles, etc., for example, the director of CDER resigned. The director of CDER and the director of CBER had announced that they would jointly create a new hub, but one of them quit. An article states that this is due to the change in administration.

Alternatively, the HHS Office of Observations reports that three of the 24 items approved under the expedited approval system have operations that differ from the normal protocol, and two of those items are not already on the market. However, I have no idea if this is in any way due to the change in administration.

While there are a few news items that we can imagine those issues are signals of some sort, we do not believe that there is a single piece of information yet that can say with certainty that this is an impact from the new administration.

In conclusion, as Mr. Ito mentioned earlier, it is unknown to us, and I don't think any company has an answer to this question.

Muraoka [M]: I understand. Thank you very much.

Moderator [M]: Thank you very much.

Mr. Barker, could you please ask a question?

Yamakita [Q]: My name is Yamakita from Jefferies. Let me ask you two questions as well.

Support

Japan 050.5212.7790
Tollfree 0120.966.744

North America 1.800.674.8375
Email Support support@scriptasia.com



The first, the meeting regarding JR-141 will be in the first half of the calendar year. Am I correct in understanding that the discussion on licensing out will be finalized after this meeting? I ask this because I think the contract situation will change depending on the time of application.

If so, is it correct to assume that after the meeting, the JR-141 and JR-171 discussions will probably proceed together?

Ito [A]: I think that depends on the other party, but I am certain that they will be interested in the meeting as we proceed with the negotiations.

So I guess it depends on how far it affects them. I think we will negotiate with that in mind. I think that the other company would probably be concerned about such things while negotiating.

Yamakita [Q]: Thank you very much.

The second point is about GROWJECT. You mentioned earlier that you are acquiring more new patients than your market share. I think you briefly mentioned the 50% target last time. Are you likely to be able to achieve this target? If so, when do you think you will be able to achieve this? Also, what are the risks, if any, with respect to inventory levels?

Ito [A]: Regarding your first question, we probably mentioned that we wanted to achieve a 50% market share through sales. We feel that this is a long-term goal, and our current situation is that we would like to start with 45% as a "Merkmal."

In this context, it is very important how to acquire new patients. I think the fact that we have achieved the 48% figure at the moment is one good signal.

The next thing we need to think about is how we can expand our business by offering a variety of products and services. Regarding this, the number of accounts has increased, which makes our business more appealing, and the way our sales force moves has changed considerably. Therefore, we would like to first firmly achieve the plan for this fiscal year, and then, based on that, examine how far we can go in the next fiscal year.

Yamakita [Q]: Is there anything we should be concerned about regarding stock?

Ito [A]: What stock are you talking about?

Yamakita [Q]: The product between the sale at the end and the shipment from your company, the hospital and the wholesaler.

Ito [A]: Distributor's stock .

Yamakita [Q]: Yes. Distributor's stock.

Ito [A]: At the moment, I don't see any strange movement in shipping and so-called consumption. I don't think those points are problematic.

Yamakita [M]: I understand. Thank you very much. That's all from me.

Moderator [M]: Thank you very much.

Mr. Maeda, could you ask a question?

Maeda [Q]: Thank you for your help. My name is Maeda from Nomura Securities. I would like to ask one point.

Support

Japan 050.5212.7790
Tollfree 0120.966.744

North America 1.800.674.8375
Email Support support@scriptasia.com



According to today's press release from SanBio Company Limited, I believe that an agreement has been signed between your company and SanBio for the trial production of AKUUGO for commercial manufacturing.

From what point in time did you start talking about this partnership? I would also appreciate it if you could disclose, to the extent you are able to do so, whether or not your facilities would be actually used, or whether or not the model would generate revenue for your company.

Ito [A]: I believe that the other party was considering this alliance after receiving conditional and time-limited approval. On top of that, they came to us.

As for facilities, we will first proceed with a trial production study, and at that stage, the product will be manufactured at our facilities. Since this is a trial production, we will make the product on a trial basis and examine whether it can be manufactured properly.

Maeda [Q]: I believe that for commercial manufacturing, the key point is that you are required to manufacture to meet two standards for domestic shipment. Am I correct in understanding that you will do that?

Ito [A]: Regarding that, I have heard that equivalence will be achieved with the current contractor.

Maeda [M]: I understand. Thank you very much.

Moderator [M]: Thank you very much.

Mr. Mizuno, could you please ask a question?

Mizuno [Q]: Thank you. Let me ask you two questions.

This may overlap with Hashiguchi's and Muraoka's questions. I believe that there is progress in the development of in-vivo or ex-vitro gene therapies for lysosomal disease, especially in the areas targeted by JR-141 and JR-171. Not in comparison to individual products, but in general terms, do gene therapy drugs and your company's drugs compete with each other clearly, or will they be increasingly differentiated according to patient profiles?

In addition, Takeda withdrew from the JR-141 project, and earlier you mentioned that the other party withdrew from the JR-171 project due to a decision based on strategic circumstances. Even globally, many companies seem to be pulling back from the rare disease area. Although not all; I know some companies focus on that, such as mega pharma companies.

What is your perception of the industry, as well as whether the number of such players is decreasing?

Kawata [A]: I, Kawata, will answer.

First, I believe it depends on whether it is in vivo or ex vivo.

First, as a simple answer, we believe that whether or not it can be used depends greatly on the patient's profile.

For example, in the case of gene therapy with AAV, as you can see from the information on clinical trials from other companies, for example, it is limited to severe forms, or cases positive for antibodies cannot be used.

In Hunter syndrome, for example, 60% to 70% of the patients diagnosed are the severe form. Of the patients present, those with the severe form of the disease often die earlier than those with the less severe form.

Support

Japan 050.5212.7790
Tollfree 0120.966.744

North America 1.800.674.8375
Email Support support@scriptasia.com



Therefore, we believe that the number of patients to whom gene therapy can be applied will be relatively small among the current patients.

We also believe that there are probably many cases where the diagnosis is not made at an age where gene therapy could be applied, for example, in areas such as MPS Type IIIA, where the clinical challenge is the long number of years before the disease is diagnosed.

In this light, I think that, for example, in the case of gene therapy with AAV, even if it emerges, its impact is expected to be relatively small.

In the case of ex vivo therapy, as has been done with Hurler's syndrome, for example, it can only be applied to very young patients because of the transplantation, so we believe that this will be differentiated from enzyme replacement therapy.

Also, regarding your question about whether or not many companies are pulling out, I am not sure of the overall trend. However, if we look at gene therapy as a whole, there are areas where this is no longer a dream drug. One of these is in the area of hemophilia, where after a very long time and a battle with the regulatory authorities, some products that have received approval are not selling at all.

Therefore, I feel that the question of what diseases must be treated with gene therapy is now being considered.

Mizuno [Q]: Thank you.

One more thing, I would like to get a feel for it as I know you can't give me more details. What is your impression of the licensing inquiries for your original J-Brain Cargo technology or the elemental technology such as the AAV with J-Brain Cargo, which you mentioned at the R&D briefing the other day?

Ito [A]: Thank you for your question.

Compared to a year ago, for example, I think the number of inquiries has increased. There are several companies that are interested in and considering gene therapy as well, albeit under the radar.

There are a number of companies that are interested and we are in discussions with, and again, I think the number of interested parties has increased compared to previous years.

As Mr. Sonoda mentioned at one of our briefings, we would like you to understand that we do not immediately conclude a contract with a company that has expressed interest. Also, although there are only two months left in this fiscal year, I hope to be able to give good news in the future over the next year.

Mizuno [M]: I understand very well. Thank you very much. That is all.

Moderator [M]: Thank you very much.

I regret to inform you that the end of the meeting is approaching, so the next question will be the last.

Mr. Matsubara, could you ask a question?

Matsubara [Q]: My name is Matsubara from Nomura Securities.

I would like to know about AKUUGO. You are talking about commercial production rather than consideration, but am I correct in understanding that you will use the same production method as the contractor that SanBio is currently outsourcing to?

Support

Japan 050.5212.7790
Tollfree 0120.966.744

North America 1.800.674.8375
Email Support support@scriptasia.com



Kawata [A]: Basically, since the product has already been approved, we understand that the manufacturing process itself is the same, just the manufacturing location is changed.

Matsubara [Q]: I understand.

Second, when does your company now expect to start generating revenue?

Kawata [A]: We are not yet in a position to give a specific date for monetization. Our current response is that we will explain again at the appropriate time.

Matsubara [M]: I understand. Thank you very much.

Moderator [M]: Thank you very much.

We apologize, but due to time constraints, this is the end of the Q&A session.

This concludes the conference call for the announcement of JCR Pharmaceuticals' financial results for Q3 of the fiscal year ending March 31, 2025.

Thank you all very much for your participation today.

[END]

Document Notes

1. *Portions of the document where the audio is unclear are marked with [Inaudible].*
2. *Portions of the document where the audio is obscured by technical difficulty are marked with [TD].*
3. *Speaker speech is classified based on whether it [Q] asks a question to the Company, [A] provides an answer from the Company, or [M] neither asks nor answers a question.*
4. *This document has been translated by SCRIPTS Asia.*

Support

Japan 050.5212.7790
Tollfree 0120.966.744

North America 1.800.674.8375
Email Support support@scriptsasias.com



Disclaimer

SCRIPTS Asia reserves the right to edit or modify, at its sole discretion and at any time, the contents of this document and any related materials, and in such case SCRIPTS Asia shall have no obligation to provide notification of such edits or modifications to any party. This event transcript is based on sources SCRIPTS Asia believes to be reliable, but the accuracy of this transcript is not guaranteed by us and this transcript does not purport to be a complete or error-free statement or summary of the available data. Accordingly, SCRIPTS Asia does not warrant, endorse or guarantee the completeness, accuracy, integrity, or timeliness of the information contained in this event transcript. This event transcript is published solely for information purposes, and is not to be construed as financial or other advice or as an offer to sell or the solicitation of an offer to buy any security in any jurisdiction where such an offer or solicitation would be illegal.

In the public meetings and conference calls upon which SCRIPTS Asia's event transcripts are based, companies may make projections or other forward-looking statements regarding a variety of matters. Such forward-looking statements are based upon current expectations and involve risks and uncertainties. Actual results may differ materially from those stated in any forward-looking statement based on a number of important factors and risks, which are more specifically identified in the applicable company's most recent public securities filings. Although the companies may indicate and believe that the assumptions underlying the forward-looking statements are accurate and reasonable, any of the assumptions could prove inaccurate or incorrect and, therefore, there can be no assurance that the anticipated outcome described in any forward-looking statements will be realized.

THE INFORMATION CONTAINED IN EVENT TRANSCRIPTS IS A TEXTUAL REPRESENTATION OF THE APPLICABLE PUBLIC MEETING OR CONFERENCE CALL. ALTHOUGH SCRIPTS ASIA ENDEAVORS TO PROVIDE ACCURATE TRANSCRIPTIONS, THERE MAY BE MATERIAL ERRORS, OMISSIONS, OR INACCURACIES IN THE TRANSCRIPTIONS. IN NO WAY DOES SCRIPTS ASIA OR THE APPLICABLE COMPANY ASSUME ANY RESPONSIBILITY FOR ANY INVESTMENT OR OTHER DECISIONS MADE BY ANY PARTY BASED UPON ANY EVENT TRANSCRIPT OR OTHER CONTENT PROVIDED BY SCRIPTS ASIA. USERS ARE ADVISED TO REVIEW THE APPLICABLE COMPANY'S PUBLIC SECURITIES FILINGS BEFORE MAKING ANY INVESTMENT OR OTHER DECISIONS. THIS EVENT TRANSCRIPT IS PROVIDED ON AN "AS IS" BASIS. SCRIPTS ASIA DISCLAIMS ANY AND ALL EXPRESS OR IMPLIED WARRANTIES, INCLUDING, BUT NOT LIMITED TO, ANY WARRANTIES OF MERCHANTABILITY OR FITNESS FOR A PARTICULAR PURPOSE OR USE, FREEDOM FROM BUGS, SOFTWARE ERRORS OR DEFECTS, AND ACCURACY, COMPLETENESS, AND NON-INFRINGEMENT.

None of SCRIPTS Asia's content (including event transcript content) or any part thereof may be modified, reproduced or distributed in any form by any means, or stored in a database or retrieval system, without the prior written permission of SCRIPTS Asia. SCRIPTS Asia's content may not be used for any unlawful or unauthorized purposes.

The content of this document may be edited or revised by SCRIPTS Asia at any time without notice.

Copyright © 2025 SCRIPTS Asia K.K. ("SCRIPTS Asia"), except where explicitly indicated otherwise. All rights reserved.

Support

Japan 050.5212.7790
Tollfree 0120.966.744

North America 1.800.674.8375
Email Support support@scriptsasias.com

