



JCR Pharmaceuticals Co., Ltd.

FY2025 Second Quarter Results Briefing Session

October 30, 2025

Event Summary

[Company Name]	JCR Pharmaceuticals Co., Ltd.
[Company ID]	4552-QCODE
[Event Language]	JPN
[Event Type]	Earnings Announcement
[Event Name]	FY2025 Second Quarter Results Briefing Session
[Fiscal Period]	FY2025 Q2
[Date]	October 30, 2025
[Number of Pages]	23
[Time]	15:15 – 16:08 (Total: 53 minutes, Presentation: 28 minutes, Q&A: 25 minutes)
[Venue]	Webcast
[Venue Size]	
[Participants]	
[Number of Speakers]	5
	Shin Ashida Representative Director, Chairman, President, and CEO
	Toru Ashida Director, Senior Managing Executive Officer, Sales, Executive Director, Sales Division
	Hiroyuki Sonoda, Ph.D. Director, Senior Managing Executive Officer, Research, Executive Director, Research Division
	Yoh Ito Senior Executive Officer, Corporate Strategy, Executive Director, Corporate Strategy Division
	Anne Bechet Senior Executive Officer, Executive Director, Development Division, JCR Europe B.V. General Manager, JCR USA Inc. General Manager

Support

Japan 050.5212.7790
Tollfree 0120.966.744

Email Support support@scriptsasia.com

[Analyst Names]*

Kazuaki Hashiguchi
Shinichiro Muraoka
Kota Maeda
Fumiyoshi Sakai
Miyabi Yamakita

Daiwa Securities
Morgan Stanley MUFG Securities
Nomura Securities
UBS Securities Japan
Jefferies Japan

*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

Email Support support@scriptsasia.com

Presentation

Moderator: I would like to begin the financial results presentation of JCR Pharmaceuticals Co., Ltd. for Q2 of the fiscal year 2025.

Before the briefing starts, we would like to inform viewers of some cautions. Please be aware that today's presentation may contain forward-looking statements based on current expectations, all of which are subject to risks and uncertainties. In addition, today's presentation and the materials used are intended to provide information about our business to shareholders, investors and the press. The information regarding developments and pharmaceutical products is not intended as advertising, medical advice, or the like.

Let me now introduce today's speakers. Shin Ashida, Representative Director, Chairman, President, and CEO,.. Toru Ashida, Director, Senior Managing Executive Officer and Executive Director of Sales Division. Hiroyuki Sonoda, Director, Senior Managing Executive Officer and Executive Director of Research Division. Anne Bechet, Senior Executive Officer and Executive Director of Development Division. Finally, Yoh Ito, Senior Executive Officer and Executive Director of Corporate Strategy Division. These five people will be speaking.

The documentation to be used today were posted on our website at 2:00 PM on October 30. If you need to have the documents at hand, please obtain them there.

This briefing will last approximately one hour and will include a presentation and Q&A session. Questions will be accepted after all the presentations have been completed. The Q&A session will last approximately 30 minutes.

Now, after a few words from Chairman Ashida, we will begin the presentations. Chairman Ashida, please go ahead.

Shin Ashida: Thank you, everyone, for joining us today. The Q2 results were very strong in terms of sales, and the Company has returned to normalcy after the previous term's losses. We were also able to achieve a contract amount of JPY5 billion.

We have received interest from several companies regarding the out-licensing of technologies related to J-Brain Cargo and AAV, and several agreements have already been finalized. In addition, to further expand our existing product portfolio and ensure stable revenue growth in the future, we are in the process of submitting applications for Agalsidase Beta BS in nine countries where Japanese approval can be leveraged.. Later, Dr. Sonoda will provide more details our technology,.which I believe represents a truly new approach.

In addition, the development of the JR-141 is progressing well, and out-licensing negotiations are on track. We appreciate your continued support.

Support

Japan 050.5212.7790
Tollfree 0120.966.744

Email Support support@scriptsasia.com



Overview: Consolidated Financial Results

Consolidated	FY2024	FY2025				Additional Remarks
	Q2 YTD	Q2 YTD	Year-on-year		Progress Rate	
			Difference	Ratio		
Net Sales	16,657	21,362	+4,705	+28.2%	56.5%	<ul style="list-style-type: none">Net Sales increased 28% year on year, mainly reflecting higher upfront and milestone payments from licensing agreements.Cost of Sales Ratio (excluding income from contractual payment) rose slightly due to lower capacity utilization.SG&A Expenses increased, mainly due to higher co-promotion fees in line with sales growth.R&D Expenses increased, driven by progress in overseas clinical development programs.Non-operating Income increased due to foreign exchange gains, while Non-operating Expenses decreased as foreign exchange and equity-method losses narrowed.Extraordinary Income mainly reflected gains on the sale of investment securities.
Cost of Sales	4,330	4,323	(6)	(0.1)%	52.7%	
Gross Profit	12,326	17,038	+4,711	+38.2%	57.6%	
Selling, General and Administrative Expenses	13,066	14,659	+1,593	+12.2%	54.3%	
SG&A Expenses	6,489	6,824	+334	+5.2%	56.9%	
R&D Expenses	6,576	7,835	+1,258	+19.1%	52.2%	
Operating profit	(739)	2,379	+3,118	-	-	
Non-operating Income	134	353	+218	+162.5%	-	
Non-operating Expenses	1,016	370	(646)	(63.6)%	-	
Ordinary profit	(1,621)	2,362	+3,983	-	-	
Extraordinary Income	1,065	209	(855)	(80.3)%	-	
Extraordinary Losses	0	31	+30	+15,975.2%	-	
Profit before Income Taxes	(556)	2,541	+3,097	-	-	
Income Taxes	134	830	+695	+515.1%	-	
Profit Attributable to Owners of Parent	(691)	1,710	+2,401	-	-	
Reference: R&D Expenses before Deducting Contribution Amount by Collaborative R&D Destinations	7,314	8,226	+912	+12.5%	48.1%	

Net Sales	FY2024 Q2 YTD	FY2025 Q2 YTD	Difference
Cost of Sales Ratio	26.0%	20.2%	(5.8)%
Cost of Sales Ratio *excluding income from contractual payment	25.3%	26.3%	+1.0%
R&D Expenses Ratio	39.5%	36.7%	(2.8)%
Operating Profit Ratio	(4.4)%	11.1%	+15.5%

YTD: year to date

YTD: year to date 3

Itoh: Now, I will explain the consolidated financial results for H1. Next slide, please.

This is the overall financial statement. Net sales were JPY21,362 million and operating income was JPY2,379 million, an increase in both sales and income compared to the same period last year. As Ashida mentioned, we have achieved financial results in the black.

Support

Japan 050.5212.7790
Tollfree 0120.966.744

Email Support support@scriptsasia.com

Breakdown of Net Sales (Consolidated)

(Unit: million yen)

Consolidated	FY2024	FY2025			
	Q2 YTD	Q2 YTD	Year-on-year		Progress Rate
			Difference	Ratio	
GROWJECT®	9,401	8,915	(486)	(5.2)%	50.1%
IZCARGO® *	2,845	3,354	+508	+17.9%	52.4%
TEMCELL® HS Inj.	1,521	1,582	+61	+4.0%	58.6%
Treatments for renal anemia	1,764	1,580	(184)	(10.4)%	51.0%
Epoetin Alfa BS Inj. [JCR]	962	296	(666)	(69.2)%	37.0%
Darbepoetin Alfa BS Inj. [JCR]	801	1,283	+482	+60.2%	55.8%
Agalsidase Beta BS I.V. Infusion [JCR]	714	426	(288)	(40.3)%	38.7%
Total Core Products	16,246	15,858	(388)	(2.4)%	51.0%
Income from contractual payment	15	5,015	+4,999	+31,895.0%	91.2%
Other*	395	489	+94	+23.9%	-
Total Net Sales	16,657	21,362	+4,705	+28.2%	56.5%

Additional Remarks

- GROWJECT®, IZCARGO®, and TEMCELL® HS Inj. all outperformed internal budgets and maintained strong momentum.
- GROWJECT®, volumes were flat year on year, but revenue declined following price revisions.
- Sales of the treatments for renal anemia remained aligned with the supply plans of Kissei Pharmaceutical Co., Ltd.
- Sales of Agalsidase Beta BS I.V. Infusion [JCR] remained aligned with the supply plans of Sumitomo Pharma Co., Ltd.
- Licensing revenue primarily consisted of upfront and milestone payments under existing agreements.
- Other income increased, primarily due to higher sales from the NPS program.

* Sales of IZCARGO® related to NPS is included in Other.

YTD: year to date 4

Before going into the details shown here, I would like to explain the sales of each product on the next page.

First, at the top is GROWJECT, then IZCARGO, and then TEMCELL. We are conducting sales activities for these three products, and all of them are performing well.

First let's look at GROWJECT at the top. With sales of JPY8,915 million, the annual progress rate is more than 50%. Although there was a slight decrease compared to the previous year, the sales volume was almost the same as then. This means that the impact of NHI price revision has been negative.

Next, let's look at IZCARGO. Sales were JPY3,354 million. Compared to the same period last year, this represents an increase of JPY500 million, or 18%.

Next is TEMCELL with JPY1,582 million, which is JPY61 million more than the same period last year. Progress compared to the budget has been very good.

The sales of Epoetin Alfa and Darbepoetin Alfa, which are sold by Kissei Pharmaceutical Co., Ltd., are in accordance with the supply contract with Kissei Pharmaceutical Co., Ltd.

Epoetin Alfa is down considerably compared to the previous fiscal year. The budget for Epoetin Alfa sales was originally set at less than half of the previous year's level, so it does not mean that Epoetin Alfa sales will decline that much, but rather that it is being affected by Kissei's inventory adjustment.

As for Darbepoetin Alfa, sales were JPY1,283 million, which means a gain of JPY482 million. This is an extremely large increase compared to the previous year, but since the supply plan also takes into account fluctuations in Kissei's inventory and other factors, I believe that sales themselves have remained mostly steady.

Sales of Agalsidase Beta were JPY426 million, a decrease of JPY288 million from the previous year. I have been told that Sumitomo Pharma's sales have not declined to this point, and that the sales themselves have remained steady. Therefore, we hope you understand that this is also due to inventory adjustments and other factors.

Support

Japan 050.5212.7790
Tollfree 0120.966.744

Email Support support@scriptsasias.com

Total pharmaceutical sales for the above totaled JPY15,858 million. Compared to the previous year, the amount is down JPY388 million.

Revenue from contracts was JPY5,015 million, a large increase compared to the previous year. As we have already announced in press releases, we have received contract revenue from licensing out gene therapy technology to Alexion, the achievement of milestones in the joint research programs we have already signed in the past, the licensing out of J-Brain Cargo technology to Acumen, domestic joint development with MEDIPAL HOLDINGS, and overseas out-licensing agreements with them.

Other sales is a positive JPY94 million, due to an increase in sales from the NPS program of IZCARGO.

Sales totaled JPY21,362 million, a JPY4,705 million increase compared to the previous year.

Now let me go back and explain the contents of the financial statements.

Overview: Consolidated Financial Results

Life is Rare 

Consolidated	FY2024	FY2025			
	Q2 YTD	Q2 YTD	Year-on-year		Progress Rate
			Difference	Ratio	
Net Sales	16,657	21,362	+4,705	+28.2%	56.5%
Cost of Sales	4,330	4,323	(6)	(0.1)%	52.7%
Gross Profit	12,326	17,038	+4,711	+38.2%	57.6%
Selling, General and Administrative Expenses	13,066	14,659	+1,593	+12.2%	54.3%
SG&A Expenses	6,489	6,824	+334	+5.2%	56.9%
R&D Expenses	6,576	7,835	+1,258	+19.1%	52.2%
Operating profit	(739)	2,379	+3,118	-	-
Non-operating Income	134	353	+218	+162.5%	-
Non-operating Expenses	1,016	370	(646)	(63.6)%	-
Ordinary profit	(1,621)	2,362	+3,983	-	-
Extraordinary Income	1,065	209	(855)	(80.3)%	-
Extraordinary Losses	0	31	+30	+15,975.2%	-
Profit before Income Taxes	(556)	2,541	+3,097	-	-
Income Taxes	134	830	+695	+515.1%	-
Profit Attributable to Owners of Parent	(691)	1,710	+2,401	-	-
Reference: R&D Expenses before Deducting Contribution Amount by Collaborative R&D Destinations	7,314	8,226	+912	+12.5%	48.1%

Additional Remarks

- Net Sales increased 28% year on year, mainly reflecting higher upfront and milestone payments from licensing agreements.
- Cost of Sales Ratio (excluding income from contractual payment) rose slightly due to lower capacity utilization.
- SG&A Expenses increased, mainly due to higher co-promotion fees in line with sales growth.
- R&D Expenses increased, driven by progress in overseas clinical development programs.
- Non-operating Income increased due to foreign exchange gains, while Non-operating Expenses decreased as foreign exchange and equity-method losses narrowed.
- Extraordinary Income mainly reflected gains on the sale of investment securities.

Net Sales	FY2024 Q2 YTD	FY2025 Q2 YTD	Difference
Cost of Sales Ratio	26.0%	20.2%	(5.8)%
Cost of Sales Ratio *excluding income from contractual payment	25.3%	26.3%	+1.0%
R&D Expenses Ratio	39.5%	36.7%	(2.8)%
Operating Profit Ratio	(4.4)%	11.1%	+15.5%

YTD: year to date 3

Sales were JPY21,362 million, as I just mentioned. The cost of sales was JPY4,323 million. The cost to sales ratio (excluding contracts), in the second row of the table at the bottom right, shows a 1% increase in the cost to sales ratio compared to the previous year, to 26.3%. As noted in the explanation on the right, this is due to a decline in factory utilization rates.

Gross profit was JPY17,038 million after subtraction, a JPY4,711 million increase from the same period last year. Selling, general and administrative expenses were JPY14,659 million, which means an overall increase of JPY1,593 million.

Within that, selling, general and administrative expenses totaled JPY6,824 million, up JPY334 million from the same period last year, mainly due to an increase in commissions paid to joint sales partners in line with higher sales. R&D expenses totaled JPY7,835 million, up JPY1,258 million from the same period last year. This is mainly due to progress in clinical development overseas, which will be explained later.

Operating income was JPY2,379 million, which means an increase of JPY3,118 million compared to the same period last year. Non-operating income was JPY353 million, mainly due to an increase in foreign exchange

Support

Japan 050.5212.7790
Tollfree 0120.966.744

Email Support support@scriptsasia.com

 **SCRIPTS**
Asia's Meetings, Globally

gains. Non-operating expenses, shown below that, amounted to JPY370 million, down JPY646 million from the same period last year, because no foreign exchange losses were recorded this time. Another reason for the decrease was lower equity in losses of affiliates.

Ordinary income was JPY2,362 million. Extraordinary gains were due to the sale of shares. The bottom line shows that net income for H1 of the current fiscal year was JPY1,710 million, a JPY2,401 million increase compared to the same period last year.

Financial Status (Consolidated)

Life is Rare 

(Unit: million yen)

	End-Mar. 2025	End-Sep. 2025	Change • Main Increase/decrease		End-Mar. 2025	End-Sep. 2025	Change • Main Increase/decrease
Current assets	51,056	55,833	+4,776	Current liabilities	43,988	47,508	+3,520
			<ul style="list-style-type: none"> Cash and deposits +2,425 Accounts receivable - trade, and contract assets +1,409 Inventories +1,655 				<ul style="list-style-type: none"> Short-term borrowings +2,400 Accounts payable - trade +462
				Non-current liabilities	13,431	13,028	(402)
Non-current assets	53,798	53,221	(576)	Total liabilities	57,420	60,537	+3,117
				Total net assets	47,435	48,517	+1,082
							<ul style="list-style-type: none"> Dividends paid (1,220) Net Profit +1,710
Total	104,855	109,055	4,199	Total	104,855	109,055	4,199
Additional Remarks					End-Mar. 2025	End-Sep. 2025	
<ul style="list-style-type: none"> Inventories increased, reflecting higher stocks of investigational products for ongoing development programs. Short-term borrowings increased to fund higher working capital needs. 				Equity ratio	44.8%	44.1%	

5

Next page, please. This is the balance sheet. Total assets amounted to JPY109,055 million, an increase of JPY4,199 million from the end of March. Total net assets, shown on the right, were JPY48.5 billion, and the equity ratio was 44.1%.

Current assets totaled JPY55,833 million, an increase of JPY4,766 million, of which cash and deposits increased JPY2,425 million, accounts receivable increased JPY1,409 million, and inventories increased JPY1,655 million. Fixed assets totaled JPY53,221 million, down JPY576 million. This is mainly due to depreciation.

On the right-hand side is shown current liabilities, at JPY47,508 million, after an increase of JPY3,520 million, and short-term borrowings, which have increased by JPY2,400 million. This is mainly due to an increase in working capital. Accounts payable increased by JPY462 million. Fixed liabilities are JPY13,028 million, which means that total liabilities are JPY60,537 million, an increase of JPY3,117 million.

One thing I forgot to mention is that inventories increased. This is due to an increase in inventories of investigational drugs for each development project.

Support

Japan 050.5212.7790
Tollfree 0120.966.744

Email Support support@scriptsasia.com

 **SCRIPTS**
Asia's Meetings, Globally

Agalsidase Beta BS I.V. Infusion [JCR]

- Biosimilar therapeutic for Fabry disease
- Commercially available in Japan since 2018

An exclusive licensing agreement for marketing authorization and commercialization was signed with Menagen Pharmaceutical Industries LLC, covering nine MENAT markets *

Menagen will file local applications in the licensed territories across the MENAT markets, leveraging the product's Japanese approval

* The Kingdom of Saudi Arabia, United Arab Emirates, the Sultanate of Oman, the State of Kuwait, the State of Qatar, the Kingdom of Bahrain, the Republic of Türkiye, the Republic of Iraq, and the Arab Republic of Egypt

6

Next page please. As Chairman Ashida mentioned, we have concluded an exclusive license agreement with Menagen Pharmaceutical Industries for the development and marketing of Agalsidase Beta BS in nine countries throughout the Middle East, Turkey and North Africa.

Agalsidase Beta BS is a biosimilar for Fabry disease and has been available in Japan since 2018. We have leveraged the product's approval in Japan to gain approval in nine countries and have Menagen Pharmaceutical carry out sales activities. We have been saying for some time that we consider stabilizing our revenue base to be a challenge, and this new move is one of the measures we have taken.

That concludes my explanation. Thank you very much.

Support

Japan 050.5212.7790
Tollfree 0120.966.744

Email Support support@scriptsasia.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"> On track for ~FY2027: Approval in US, EU, Brazil
JR-142	Pediatric GHD	Ph3 (Japan)				<ul style="list-style-type: none"> Patient recruitment is on track
JR-171	MPS I (Hurler syndrome etc.)	Global Ph1/2 completed				<ul style="list-style-type: none"> Partnering activities ongoing
JR-441	MPS IIIA (Sanfilippo syndrome type A)	Ph1/2 (Germany)				<Ph1/2> <ul style="list-style-type: none"> Achieved 1-year clinical data for the initially planned dose groups
		Ph1 (Japan)				<Ph1> <ul style="list-style-type: none"> Patient enrollment completed
JR-446	MPS IIIB (Sanfilippo syndrome type B)	Ph1/2 (Japan)				<ul style="list-style-type: none"> Recruitment of first cohort completed Partnering with MEDIPAL HOLDINGS
JR-471	Fucosidosis					<ul style="list-style-type: none"> Partnering with MEDIPAL HOLDINGS
JR-479	GM2 gangliosidosis (Tay-Sachs disease, Sandhoff disease)					<ul style="list-style-type: none"> Partnering with MEDIPAL HOLDINGS

8

Bechet*: Good afternoon, and thank you for joining us today.

My name is Anne Beche and I will give a brief overview about the progress of our development assets.

Of course, first, a brief overview about our JR-141 asset for MPS II. Our Global Phase 3, after having completed recruitment in July, continues to progress as per plan.

We continue our interactions with regulators and are exactly on track for a target approval in fiscal year Japanese 2027. We are also proceeding with our JR-142 for pediatric growth hormone with the patient recruitment progressing as previously indicated.

Our MPS I asset for Hurler, named JR-171, has now completed the phase1/2. The partnering activities are currently ongoing. JR-441, which is our asset for MPS IIIA, is progressing well with the trial in Germany, so a phase1/2 study that has now achieved the first year of clinical data for the initially planned dose groups. We have also initiated a phase1 in Japan with a patient enrollment that is completed.

JR-446, our asset partnered with MEDIPAL HOLDINGS for MPS IIIB, is progressing on track with the recruitment of our first cohort currently completed.

And next to those assets that are currently in clinical phase, we have JR-471 for Fucosidosis partnered with MEDIPAL HOLDINGS, progressing at present in preclinical, as well as JR-479 for GM2, also partnered with medical holdings.

We will target to transition these compounds in clinical as soon as feasible. Thank you very much.

Support

Japan 050.5212.7790
Tollfree 0120.966.744

Email Support support@scriptsasia.com



Difficulty in delivery to target tissues

- Central nervous system, muscle, cartilage, etc.
- AAVs do not cross the blood-brain barrier¹



Safety issues

- Acute liver toxicity, thrombotic microangiopathy, neurotoxicity²
- Deaths due to liver injury have occurred in clinical trials²⁻⁴



Large-scale production of AAV vector

- Complex manufacturing process, requiring advanced technology⁵
- Quality control is extremely important



Neutralizing antibodies

- Risk of pre-existing antibodies making patient ineligible for AAV-mediated gene therapy⁶



Cost of treatment

- Single-treatment solution, but at a high cost⁷

¹. Dadi R, et al., *Int J Mol Sci.* 2024; 25(2): 1050. ². Wang JH, et al., *Signal Transduct Target Ther.* 2024; 9(1): 78. ³. Duan D. *Mol Ther.* 2023; 31(11): 3123-3126. ⁴. *Nat Biotechnol.* 2020; 38(8): 910.
⁵. Jiang Z, et al., *Trends Biotechnol.* 2023; 41(10): 1268-1281. ⁶. Weber T. *Front Immunol.* 2021; 12: 658399. ⁷. Kliegman M, et al., *Nature.* 2024; 634(8033): 307-314.

Sonoda: Hello everyone. My name is Sonoda. I would now like to explain the progress in JCR's proprietary gene therapy technology, JUST-AAV, our gene therapy platform.

As I think we have explained in the past, the adeno-associated virus (AAV) in use today is recognized as a very promising treatment tool, but it still has many issues that need to be addressed and further improved.

We've been working on this for many years, and we have the top two issues mentioned here, AAV with increased efficiency of delivery to the target tissue, and AAV with reduced concerns about side effects. We have put together this explanation.

JUST-AAV



AAV: Adeno-Associated Virus

JCR

Ultimate destination of organ

Safeguarding against off-target delivery

Transformative technology

**AAV with directionality to target tissues/organs
and reduced accumulation to specific tissues/organs**

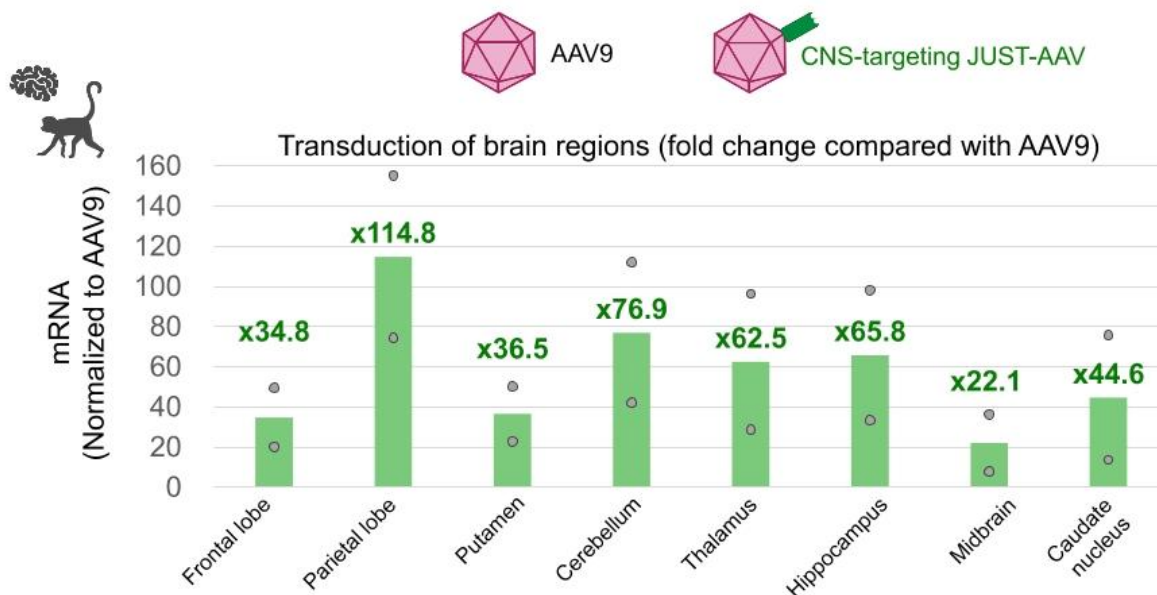
ex. CNS,
Muscle

ex. Liver

11

This is what JUST-AAV is all about. JUST, which is the acronym for the wording here, is a way to increase directionality to the target tissue and reduce the accumulation to tissue you don't want to deliver to, which in this case is mainly the liver. We have called this proprietary technology of ours, JUST-AAV.

Evaluation of CNS-targeting JUST-AAV Delivery Efficiency



The 32nd Annual Congress of the European Society of Gene and Cell Therapy (ESGCT2025)

CNS-targeting JUST-AAV: n=2, AAV9: n=1
GOI: H2B-HA, 2.7E13 VG/kg, 4 weeks after admin

12

This is a CNS-targeting type. This shows data on monkeys with AAVs that have been engineered to go to the brain more. The vertical axis shows the messenger RNA expression level. And the horizontal axis shows the parts of the brain. How much has the expression efficiency increased at each part?

Support

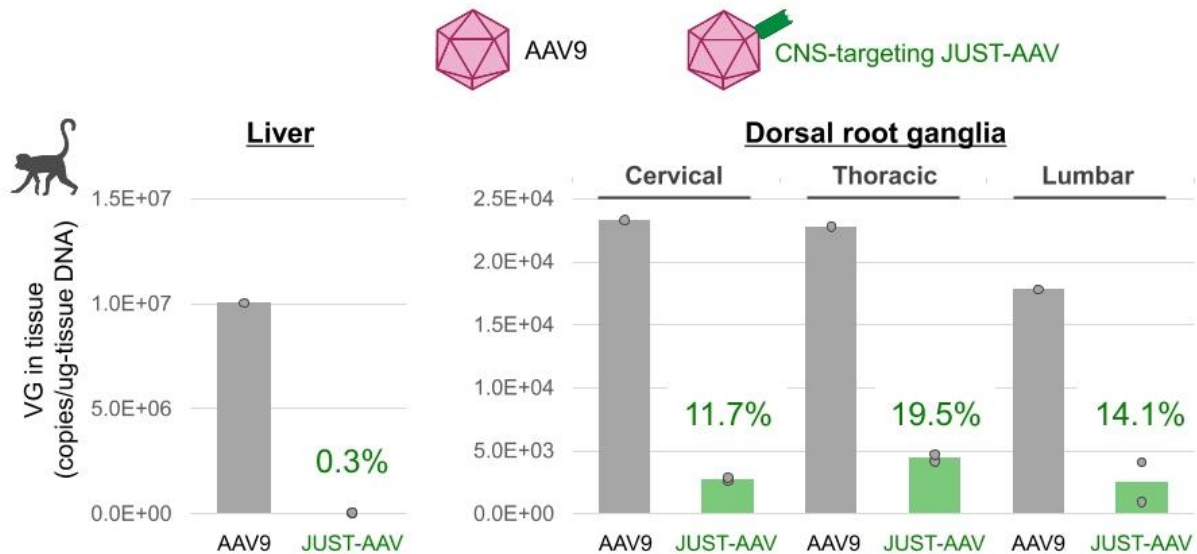
Japan 050.5212.7790
Tollfree 0120.966.744

Email Support support@scriptasia.com

You can see the data is shown as a comparison with serotype9, commonly used AAV. The data shows how many times the expression rate of messenger RNA is improved when AAV9 is set as 1. As shown here, each part shows improvement in expression from various multiples of ten to more than 100-fold.

Evaluation of Tropism Reduction for Specific Tissues

Life is Rare 



The 32nd Annual Congress of the European Society of Gene and Cell Therapy (ESGCT2025)

CNS-targeting JUST-AAV: n=2, AAV9: n=1
GOI: H2B-HA, 2.7E13 VG/kg, 4 weeks after admin

13

The graph here refers to safety. It shows how much distribution has been lowered for organs that we do not want to deliver to. This is also compared with AAV9. The gray bar is AAV9, whereas the green bar is JUST-AAV, indicating how much the distribution has decreased.

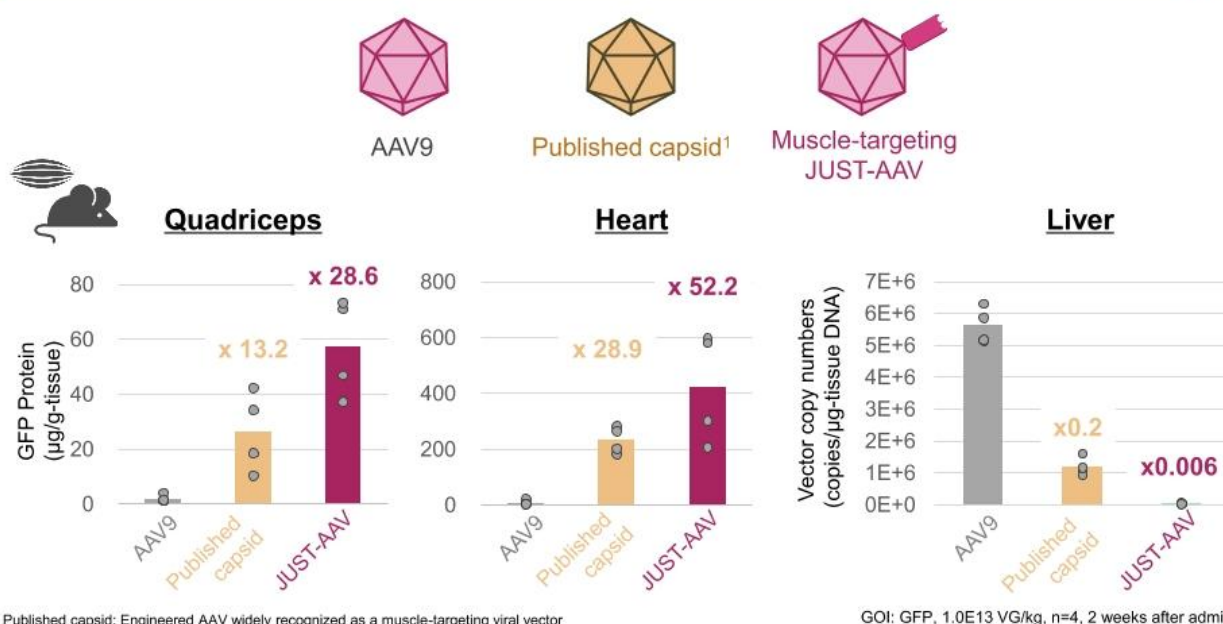
This is data from monkeys. The left side is for the liver and the right side is dorsal root ganglia data. As you can see here, reduction of more than 99% is already visible for the liver, and we have also succeeded in greatly reducing distribution in the dorsal root ganglia.

Support

Japan 050.5212.7790
Tollfree 0120.966.744

Email Support support@scriptsasia.com

 **SCRIPTS**
Asia's Meetings, Globally



The next graph shows tissues other than the brain. In this case, we are showing data on muscle tissue. We believe that the advantage of our JUST-AAV is that we can design for the AAV. That design can be for CNS-targeting, or to make it harder to deliver to the liver, or mixing those designs together. We think that is our advantage with this technology, plus we are showing here that we have successfully developed a Muscle-targeting AAV.

This is mouse data. The two graphs to the left are for muscles. The quadriceps and the heart. On the right is liver data. This is also shown in comparison to the commonly used AAV9. The bars marked "published capsid" in the middle in yellow are well-known AAVs that have been declared to be highly muscle tropism, and we are comparing them side by side as a milestone. Compared to this, the data verifies the superiority of our Muscle-targeting JUST-AAV.

In this graph, the high bars show effective transfer to that tissue. If you look at the quadriceps muscle, on the left, and the heart, in the middle, you can see that AAV9 is 1, so there are almost no bars. Compared to that, the quadriceps muscle and the heart have 28 times and 52 times higher expression levels, respectively.

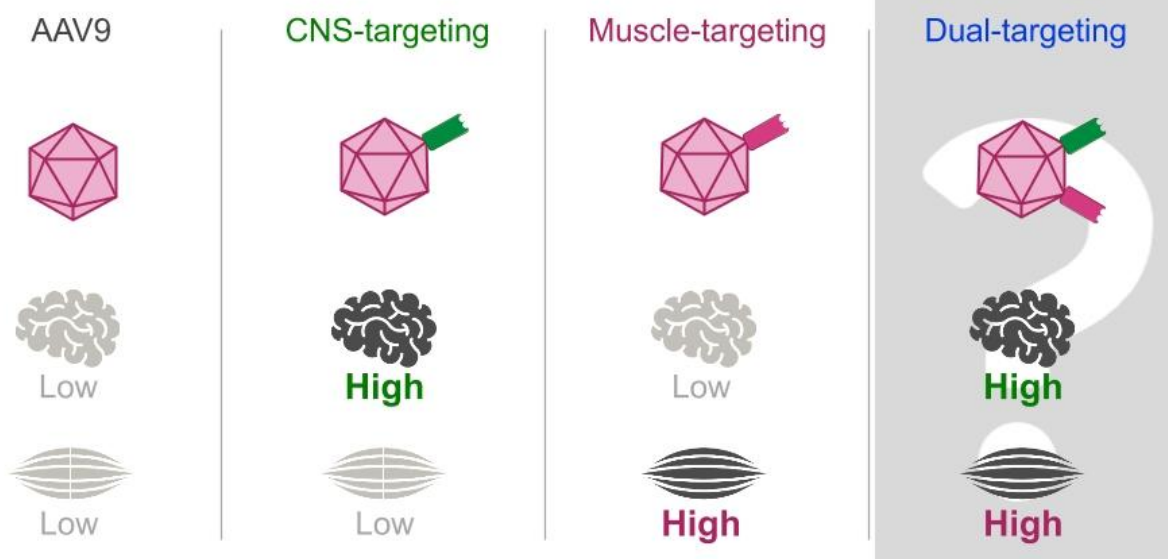
You can also see that our Muscle-targeting JUST-AAV has a higher expression level than the yellow, published capsid bar in the middle.

And there is also the safety aspect. If you look at the liver data, you will see that the liver accumulation of JUST-AAV is extremely low. Therefore, we believe that we have created a new AAV that meets the needs of the current clinical problems by increasing transferability to the muscle and greatly reducing liver tropism.

Support

Japan 050.5212.7790
 Tollfree 0120.966.744

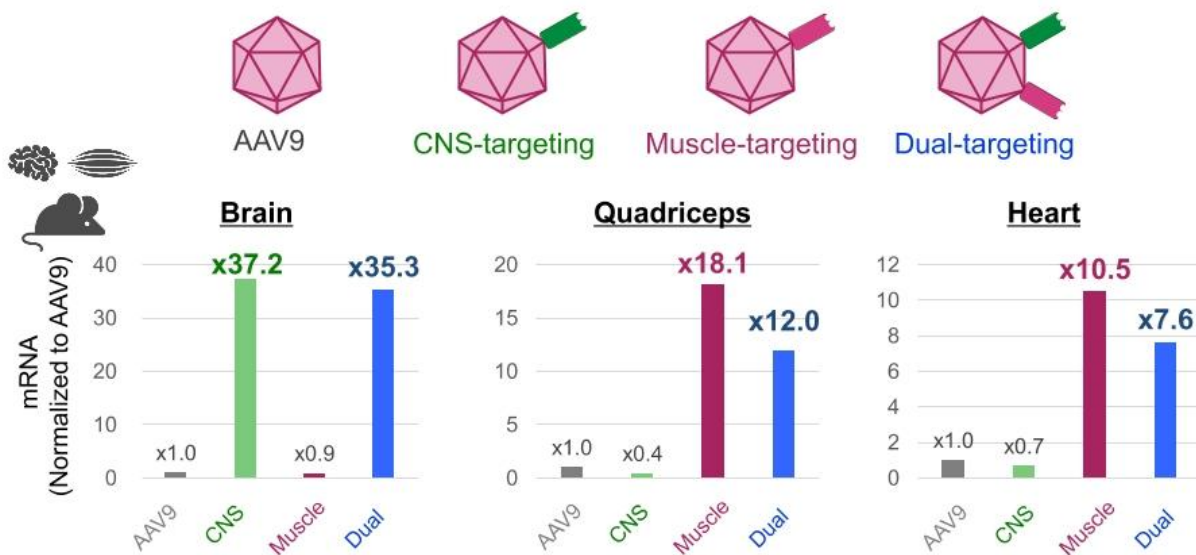
Email Support support@scriptsasia.com



15

Next, as I mentioned earlier, in terms of whether it is designable or not, we confirmed that it can target both CNS-targeting and Muscle-targeting types, if you add them together.

Exploring the Promise of Dual-targeting JUST-AAV



The 32nd Annual Congress of the European Society of Gene and Cell Therapy (ESGCT2025)

Barcode study (AAV pool was injected)
5.0E12 VG/kg iv per each AAV, n=1, 2 weeks after admin

16

This is mouse data. Here we compare the AAV9 with a CNS-targeting one, a Muscle-targeting one, and one that combines both types, Dual-targeting.

The tissues we are looking at here are, from left to right, the brain, the quadriceps, and the heart. I hope you can see that the first one, on the left, focuses on CNS tissue and the two on the right focus on muscle tissue. It is a little difficult to see, but I hope you can distinguish them by the color of the bar.

Support

Japan 050.5212.7790
Tollfree 0120.966.744

Email Support support@scriptasia.com

This is also compared to AAV9. The green bar is a CNS-targeting AAV, so it goes to the brain very well. In muscle, the level is roughly the same compared to AAV9. In other words, it goes to the brain well, but not so much to the muscles.

Next is the pink bar. This is muscle-directed, so in the brain it is at the same level as AAV9, but in the muscles and heart it shows a very high distribution.

The blue bar is a combine two types, Dual-targeting type. In this, we are showing a high configuration both in the brain and in the muscles, which means that we are achieving what we set out to do.

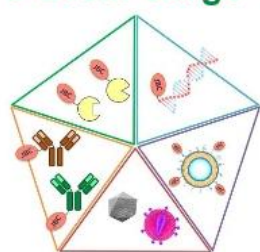
Our JUST-AAV system is designed to create tags, binders, and other such items that can target tissue, and then place them on our system to display them on the surface of the AAV, and then provide tissue-targeting according to the tags displayed. So, as in the case of this Dual-targeting, by replacing the tag or adding a type of tag, we can determine what we want to carry to what tissue. I think the best thing about it is that it allows us to design.

Shaping the Future with JCR's Proprietary Technologies

Life is Rare 

Partnering our groundbreaking technologies and creating breakthrough therapies in various disease areas beyond rare

J-Brain Cargo®



Blood-Brain Barrier transport applicable to various modalities

JUST-AAV



AAV with enhanced delivery to target tissues and reduced liver tropism

Lysosomal Storage Disorders

Neurodegeneration

Muscular Diseases

Neuroinflammation

Neuro-oncology

17

We have been talking about applying J-Brain Cargo to various modalities and delivering it to the brain. One of these modalities is AAV, and we have been focusing on AAV and have created a technology called JUST-AAV.

As I have just shown, with JUST-AAV, we have created a technology that can target tissues other than the brain. We did that by applying J-Brain Cargo, which is technology that targets the brain, to non-brain targets. By combining this technology with our original AAV technology, we have created a gene therapy tool that can deliver efficiently to tissues other than the brain. We believe that the same tool can be used for different modalities in the future. In other words, I believe that multiple target systems for multiple modalities, and the mixing of these systems, can cover many disease areas.

It is good to be able to do various things like this, of course, but there is a limit to the areas of disease that can be covered by one company. We would like to apply this technology and collaborate with various other companies and firms to make it the gold standard. That is all. Thank you very much.

Support

Japan 050.5212.7790
Tollfree 0120.966.744

Email Support support@scriptasia.com

 **SCRIPTS**
Asia's Meetings, Globally

15

Moderator: Thank you for your attention.

Support

Japan 050.5212.7790
Tollfree 0120.966.744

Email Support support@scriptsasia.com



Question & Answer

Moderator [M]: We will now move on to the question-and-answer session. We will take questions first from analysts and then from the media. Please note that each person is limited to two questions, in a one-question, one-answer format, but that you may raise your hand again if you have further questions.

We will now begin the question-and-answer session. Mr. Hashiguchi, please go ahead.

Hashiguchi [Q]: Daiwa Securities, this is Hashiguchi. Thank you for taking my questions. In his opening remarks, I believe that Chairman Ashida mentioned that negotiations for the out-licensing of JR-141 are progressing well. I would like to know how much progress has been made at this point and what kind of timing your company is aiming for in reaching an agreement.

Phase III study is already in progress, and I think sales are a little ways off, so I don't see the need to rush too much. Meanwhile, I think you need to decide on JR-171 sooner rather than later in terms of moving on to the next step, but I don't think you mentioned earlier the status of negotiations on the out-licensing of JR-171. I would appreciate an update on this situation as well.

Ito [A]: I will answer this question. Regarding the out-licensing of JR-141, we are in the process of discussing this. However, at this point, it is too early to say when the contract will be signed. We will try to bring you good news as soon as possible.

Next, regarding JR-171, we have been in negotiations with several companies, and some of them are interested in the project. However, as was the case last time, we do not have anything new to report this time. That is all.

Hashiguchi [M]: Thank you. That is all.

Moderator [M]: Thank you very much. Next is Mr. Muraoka. Please ask your questions.

Muraoka [Q]: I am Muraoka from Morgan Stanley MUFG Securities. As for business performance, now that you have almost achieved the contract amount and the annual plan for operating income of JPY2.6 billion, how should we expect Q3 and Q4 to be if things continue as they are? It's difficult to achieve annual profit if you don't break even, but I think there is a high probability that Q3 and Q4 will be in the red if there is not much more contract money to come in. It would be helpful if you could guide us on how we should think about Q3 and Q4.

Ito [A]: I will answer. Regarding that, as you mentioned, contract revenue is also budgeted at JPY5.5 billion, so we are making good progress at JPY5 billion.

As for operating income, at this time, I cannot give you specific figures for Q3 and Q4, but we have not revised our targets for the full year, so I think we are moving steadily toward achieving these targets.

However, product sales were strong in H1, and if we can show similar progress in H2, we believe that we will be able to increase our profit as well.

Muraoka [Q]: Thank you very much. In terms of contract funds and the budget, there is still JPY500 million left, but this remaining JPY500 million was a fairly predictable figure, and I think that is how it was structured. Is that understanding correct? If possible, I would appreciate it if you could tell me at what point it becomes a Q3 or a Q4 issue.

Support

Japan 050.5212.7790
Tollfree 0120.966.744

Email Support support@scriptasia.com

 **SCRIPTS**
Asia's Meetings, Globally

Ito [A]: As mentioned about contract revenue at the previous briefing, or the one before that, we have prepared the budget based on the firm expectation that we will be able to record it.

In that sense, I cannot say now when the timing is right, but we hope to achieve contract income within the next six months.

Muraoka [Q]: Thank you very much. Sorry, I have just one question about JUST-AAV. I have not studied this enough, but when delivering this to the brain or to the muscles, is this tag part not transferrin, but a completely different molecular substance? Or do you add something to the transferrin? Sorry, but I don't understand the fundamentals at all.

Sonoda [A]: Thank you very much. I am Sonoda. I will answer your question. We just presented at a conference the other day, so I don't think you don't know enough.

The CNS target is a transferrin receptor, but we have not yet disclosed the muscle target. We have found a new target and we are using that. That concludes my answer.

Muraoka [Q]: I understand. Can this be found by looking up the patent?

Sonoda [A]: Yes. Of course it is written in the patents, but I don't think they are open yet.

Muraoka [M]: I understand. Thank you. That is all.

Moderator [M]: Thank you very much. Next, Mr. Maeda, would you please ask your questions.

Maeda [Q]: Thank you. I am Maeda from Nomura Securities. First of all, I would like to ask about the status of the contract with SanBio Co, Ltd. I remember that you have a contract manufacturing agreement in place, but I don't think you have a definitive agreement yet. Could you please tell us your current thoughts about the timing of moving that discussion forward?

Ito [A]: I will answer this question as well. We have concluded a trial manufacturing contract with SanBio, and we are now in the process of confirming whether we can manufacture the product properly.

If this turns out to be successful, we will sign a contract for commercial production and actually manufacture the product. However, in that case, it would mean an additional manufacturing site for SanBio, so it would be necessary for application for partial changes to approved matters, and we cannot say at this stage when the commercial production contract will be signed. However, we are steadily advancing with the contract for trial production.

Maeda [Q]: I understand. Thank you. Next question is about biosimilars. At the most recent meeting of the Central Social Insurance Medical Council, there was some talk about applying the G1/G2 rule to original biologics. What do you think will be the impact on the volume or price of your biosimilars in the future? I would like to know your thoughts at this moment.

Ito [A]: I will answer your question. I cannot give an immediate answer as to how it will affect us, but if there is any recent change in the situation of our biosimilars, it is not about the drug price, but about how it will be affected by Chugai's announcement that it will discontinue the production of Epogin. What is the impact of this change? We can assume that the demand for our Epoetin alfa will increase, and we are also thinking that it may have an impact on Darbepoetin alfa.

Maeda [M]: Thank you very much. That's all from me.

Moderator [M]: Thank you very much. Now, Mr. Sakai, please ask your questions.

Support

Japan 050.5212.7790

Tollfree 0120.966.744

Email Support support@scriptsasia.com

Sakai [Q]: My name is Sakai, from UBS Securities. I'm sorry to be persistent on the contract money, but I'd like to clear something up.

I believe that regarding the contract fee with Alexion, the upfront payment was already budgeted for this term, and that this has been received or recorded up to Q2 of this year. I don't think you will say what will happen in Q3 and Q4, but I believe that the license option agreement for Acumen and J-Brain Cargo has been signed. I guess it depends on when the option contract will take effect for this part, but it is my understanding that it is no longer included in the budget for this fiscal term, nor is JR-171. Is that correct?

Ito [A]: I will answer this question. JR-171 is not included in the budget this time. Also, the option agreement with Acumen and what happens if it is exercised is not included in the budget for this fiscal term.

Sakai [Q]: So, the option contract is disclosed, but as to when it will be enforced, do you have some sort of time frame set?

Ito [A]: I cannot mention it at this time, but we have an assumption.

Sakai [Q]: I understand. Your answer is that you have not budgeted on that basis.

Ito [A]: That's right.

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

Moderator [M]: Thank you very much. The next question is from Yamakita-san. Please go ahead.

Yamakita [Q]: My name is Yamakita from Jefferies Securities. My first question is about Izcargo. I think the total number of cases administered remained flat QoQ this time, but there was talk last year that the administration time would be shortened and then the number of cases are increased, so is it safe to assume that the impact of that has run its course?

Toru Ashida [A]: Thank you for your question. We believe that the impact of the shortening of administration time has had a very positive effect this quarter.

In this H1, two patients were newly diagnosed and four patients were switched from other drugs, so six cases were administered in H1, which we regard as excellent.

Yamakita [Q]: I understand. Thank you. What are some of the factors that led to the flatness in Q2? Was it due to the increase in Q1?

Ashida Toru [A]: Regarding this, we will follow up with existing patients receiving the drug, and since administration to patients receiving the drug in H1 of the fiscal year was slightly faster than we had expected, we are determined to complete the budget and forecast for the current fiscal year.

Yamakita [Q]: I understand. Thank you. Second, I would like to ask about research and development expenses. I think you said that progress in overseas clinical trials means they are increasing and that the increase is in line with the expected level, but is there any temporary factor involved? What I am wondering is, what does not go into Q3 and Q4? For example, you mentioned earlier that inventory has increased a little, but could you tell us if there were any factors such as temporary manufacturing costs?

Ito [A]: Thank you for your question. Regarding your question about R&D expenses, there was no such factor as a one-time increase in H1.

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

Support

Japan 050.5212.7790

Tollfree 0120.966.744

Email Support support@scriptsasia.com

Moderator [M]: Thank you very much. Next to ask a question is Mr. Maeda. Please go ahead.

Maeda [Q]: Thank you for calling on me again. In the JUST-AAV project, you have found a new target for delivery to muscle cells, but is it possible to deliver separately to the heart and other muscles?

Sonoda [A]: I will answer this. At this time we are unable to separate these. I believe that if we try to separate them according to those needs, it is possible to find a target. However, at this point in time, we have developed a technology to target muscle, including cardiac muscle, so at this point in time, the data I have presented today shows that distribution occurs at the same level to cardiac muscle and skeletal muscle.

Maeda [Q]: I see. In fact, do the conditions required by the parties with whom you negotiate licenses have to be cardiac-specific?

Sonoda [A]: That's on a case-by-case basis. There is, of course, a need to deliver to the heart. However, if there is a case where we do not want to delivery to the skeletal muscle, I think we need to aim for a heart-only version, but there have not been many cases like that so far. Of course there are cases where they want to deliver to the heart, but I don't think there are too many cases at the moment where they don't want to deliver to skeletal muscle, or only to the heart.

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

Moderator [M]: Thank you very much. Now, to all participants, including members of the media, do you have any questions? Yokota-san, please ask your questions.

Yokota [Q]: My name is Yokota, from Kobe Shimbun newspaper. Thank you for taking my questions. I would like to ask Chairman Ashida two questions. The first is that the Takaichi administration has come to power, and it has been promoting expansionary fiscal policy and accommodative monetary policy in particular. Is there any aspect of this area that will affect your business?

Secondly, the Nikkei Stock Average has recently been rising considerably again, and I would appreciate any thoughts you may have on shareholder return policies in relation to this.

Shin Ashida [A]: Since the Takaichi administration has been formed, we still don't know exactly what direction it will take, so we are just now watching to see what policies will emerge. The movements of our share price are not very good. I think that if we can make more contracts for our JR-141, JR-171 that was mentioned earlier, and for collaborations with other companies, or for products that use our technology, it would have a great impact on the share price.

Yokota [M]: I understand. Thank you.

Moderator [M]: Thank you very much. Next, Yamaji-san, please ask your questions.

Yamaji [Q]: Thank you. I am Yamaji from Nikkei Biotechnology & Business. I would like to ask about JUST-AAV. I understand that you intend to commercialize the Dual-targeting JUST-AAV for lysosomal diseases and neurodegenerative diseases, as shown on page 17 of the document. I wonder if it is necessary to be able to actually deliver genes to multiple organs, or if it would be possible to further enhance the efficacy of pharmaceuticals. Could you please explain the significance of the dual targeting?

Sonoda [A]: I would like to explain. That depends on the disease. For example, the dual specificity we have shown in this case is for the central nervous system (CNS), including the brain, and for muscle. Normally, it is brain and muscle, so diseases in the neuromuscular area. We can target both the nervous system and the

Support

Japan 050.5212.7790

Tollfree 0120.966.744

Email Support support@scriptsasia.com

muscles, and by delivering genes there, increase what is missing or reduce what is too much. There are many diseases that may or may not be cured by doing these things. But there are many patients like this.

Of course, there are diseases that can be cured by targeting only the brain or only the muscles, but since nerves and muscles are actually connected, there is a great possibility that effective treatment methods can be developed by targeting both. I believe that the Dual-targeting approach will greatly expand the range of diseases that can be covered.

As I mentioned, we started with the brain and muscles, but what we wanted to show today is that we have this kind of design capability, and I think that is the point where we have advantage.

If we can use specific mouse models to explain which diseases can be treated with this technology, which would be helpful for you to understand more. We are actually conducting trials of such things at the research level, so some time, in response to the question you just asked, I hope to be able to show that there are actual examples of such things and that they can be improved to this extent.

Yamaji [Q]: Thank you very much. I would like to ask one more question. Are there any other diseases or ailments that you want to target by using this bi-specific JUST-AAV in new development?

Sonoda [A]: Of course there are. However, since this is a strategic matter for us, we cannot say at this moment what that disease is, but we would like to determine exactly which diseases we should collaborate with outside companies and outside parties for, and which diseases we should work on. So, in answer to your question, yes. The answer is that of course there are.

Yamaji [M]: Thank you very much. That is all.

Moderator [M]: Thank you very much. Are there any other questions? If there are no further questions, this concludes the question-and-answer session.

This concludes the financial results briefing for Q2 of the fiscal year ending March 31, 2026, for JCR Pharmaceuticals Co., Ltd. Thank you all very much for joining us 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

Email Support support@scriptsasia.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

Email Support support@scriptsasia.com

