JCR Pharmaceuticals Co., Ltd.

Financial Results Briefing for the Fiscal Year Ended March 2024

May 13, 2024

Event Summary

[Company Name]	JCR Pharmaceuticals Co., Ltd.
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[Participants]	
[Number of Speakers]	6

Shin Ashida	Representative Director, Chairman, President, and CEO
Toru Ashida	Senior Vice President Sales Executive Director, Sales Division
Hiroyuki Sonoda, Ph.D.	Vice President, Research Executive Director, Research Division
Yoh Ito	Senior Corporate Officer Corporate Strategy Executive Director, Corporate Strategy Division
Yoshihiro Ohta	Director, Accounting Department, Corporate Strategy Division
Anne Bechet	JCR Europe B.V. General Manager

[Analyst Names]*	Hidemaru Yamaguchi	Citigroup Global Markets

Kazuaki Hashiguchi	Daiwa Securities
Fumiyoshi Sakai	UBS Securities Japan
Shinichiro Muraoka	Morgan Stanley MUFG Securities
Shinya Tsuzuki	Mizuho Securities
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.

Presentation

Moderator: We will now commence the financial results briefing of JCR Pharmaceuticals, Co., Ltd. for the fiscal year ended March 31, 2024. First, let me explain today's language settings. Please select “off”, “Japanese”, or “English” channel by clicking the translator icon at the bottom of your Zoom window.

Before we begin the briefing, I would like to say a few words to the audience. In the following discussion, we may make forward-looking statements based on our current expectations, all of which are subject to risks and uncertainties. Investors are advised in advance that actual results may differ materially from the forecasts.

Today's presentation and the material used today are intended to provide shareholders, investors, and the press with information about the Company's business. Information on developed products and pharmaceuticals is not intended as advertising, medical advice, or as a guarantee of future results or of the efficacy of products under development. This briefing is being recorded for posting on our website at a later date. Let me now introduce today's speakers. Representative Director, Chairman, President, and CEO, Shin Ashida.

Shin Ashida: Thank you very much.

Moderator: Toru Ashida, Senior Vice President Sales Executive Officer, Sales Division.

Toru Ashida: Thank you very much.

Moderator: Hiroyuki Sonoda, Ph.D., Vice President, Research Executive Director, Research Division.

Sonoda: Thank you very much.

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

Ito: Thank you very much.

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

Ohta: Thank you very much.

Moderator: Finally, Anne Bechet, JCR Europe B.V. General Manager.

Bechet: Thank you.

Moderator: These six speakers are present today. The document to be used today was posted on our website on May 13, at 11:45 AM. Please download and refer to it as necessary.

Today's presentation and Q&A session will last approximately one hour. Questions will be taken in batches after all presentations are completed. The Q&A session will last approximately 30 minutes.

We will now give an overview of the financial results and business activities. Today, after a greeting from Chairman Ashida, Anne Bechet will explain the progress of development pipelines, Sonoda will explain the next wave of technology innovations, and finally, Ito will explain the consolidated financial results for the fiscal year ended March 31, 2024.

Key Messages

Reach Beyond, Together JCR Pharmaceuticals

FY2023
Revenue and earnings growth from increasing sales of core products

FY2024
Strengthen organizational structure to support global clinical development and increase R&D investment

- Expand clinical development team and establish an integrated structure of experts in Japan, US, Europe and Brazil with JCR Europe playing a lead role
- JR-141：Complete patient enrollment for an interim analysis of the global Phase III
- JR-441：Phase I/II study on track (First patient dosed in Oct 2023)
- JR-446：Initiate Phase I/II study in 1st half of FY2024
- JR-171：Discussions on licensing-out ongoing

Shin Ashida: My name is Ashida. Thank you very much for all your help. Thank you again for your cooperation today. Today, We would like to explain the status of clinical development, new progress in research, and achievements.

Sales for the fiscal year ended March 2024 exceeded JPY40 billion for the first time. Operating profit was slightly below the forecast. We have been thinking of creating a system that allows us to conduct our own clinical development on a global basis. To do so, we invested to enhance our European, US, and Brazilian sites, and to bring in new people and set up a system that would allow us to do our own clinical development. As a result, operating profit was lower than expected.

Regarding clinical development, we currently have projects, JR-141 and JR-441, both of which are progressing very well. Anne Bechet will explain the status later. In research, we are also working on something new in addition to what we have been doing for rare diseases, which is to deliver protein products into the brain. Sonoda will explain what this is all about.

In the future, considering that we are to deal with business in the rare diseases arena as we do, it will be difficult to develop if we only look at the Japanese market. So, we would like to consider doing clinical trials globally and reaping future profits there. For this reason, we have put in place the structure that allows us to focus on global development.

Sales for the current fiscal year were JPY40 billion, but we are currently working on development to increase this to JPY70 billion or even JPY100 billion for the future. We are confident that we will be able to achieve this goal. JR-141 and JR-441 are progressing very well. Anne Bechet will give you an update on the progress later.

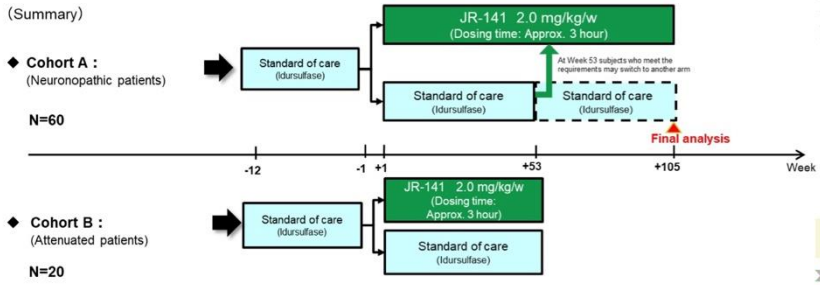
I am contemplating making this year a year for the new younger generation to participate in management for the future, and to create a new structure for the next fiscal year.

We look forward to your continued support of JCR. Thank you very much.

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

Reach Beyond, Together

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



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

Current Status

- Recruiting
- Number of Clinical trial sites (as of Apr 2024):
  - US: 5
  - Europe: 12
  - Latin America: 4

  ➡Trial is on going in 10 countries
  - Further sites to open in EU, US, Latin America to accelerate recruitment

Achievements

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

- **Completed enrollment of all 20 eligible patients in Cohort B**
- **Enrollment in Cohort A on track**

FY2023 4Q 3

Bechet*: Thank you very much Chairman Ashida. We will start with an update concerning JR-141 currently in Phase III.

As previously presented, we are targeting to reach two objectives with our Phase III study.

The first one is to demonstrate that JR-141 has a superior activity on the CNS symptoms in MPS II compared to standard enzyme replacement therapy (ERT). At the same time, we would also like to demonstrate that the control of the somatic symptoms of the disease is as good as with standard enzyme replacement therapy.

At present, we have 21 sites open spanning over Europe, USA, and Latin America, and we are targeting to activate more sites in the course of 2024 to further enhance and accelerate recruitment. We are proud to report the full enrollment of the cohort B, which is the cohort comprising the attenuated patients.

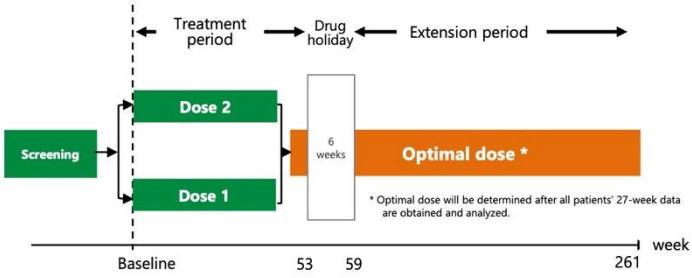
Furthermore, we anticipate at present that we will have the number of patients required for the interim analysis of the cohort A enrolled by the end of the first half of this year. We are hands -on target, and we will follow now with the next slide with an update about JR-441 or MPS IIIA asset.

JR-441 (BBB-penetrating ERT for MPS IIIA)

Reach Beyond, Together JCR Pharmaceuticals

July 2023 – Started Global Phase I/II study (JR-441-101) in Germany

JR-441-101 study overview



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

Achievements and next milestones

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

- The Phase I/II study is progressing well
- Started recruiting pediatric patients after approval from the independent data monitoring committee

FY2023 4Q 4

You can see here an overview of our global Phase I/II study designed together with expert clinicians, but also with the close involvement of parents of children affected with MPS IIIA.

Our main objectives with this study are to establish the safety profile of JR-441, as well as early pharmacodynamics in order to set up the optimal dose for the next phase of development. At present, the trial is progressing very well.

The last patient is to be enrolled before the end of the first half of 2024, and the one -year result is expected by the end of 2025.

R&D Pipeline

Reach Beyond, Together JCR Pharmaceuticals

Code	Indication	Status	Upcoming Milestones
JR-141	MPS II (Hunter syndrome)	Global Ph3	• Q1 FY2024: All patients enrolled necessary for interim analysis • ~FY2027: Approval in US, EU, Brazil
JR-171	MPS I (Hurler syndrome etc.)	Global Ph1/2 completed	• Extension study is ongoing • Licensing out under negotiation
JR-142	Pediatric GHD	Ph2 (Analysis completed)	• FY2024: Phase 3
JR-031HIE	Hypoxic ischemic encephalopathy in neonates	Ph1/2 (Analysis completed)	• TBD
JR-441	MPS IIIA (Sanfilippo syndrome type A)	Global Ph1/2	• 1st Half FY2024: LPI • 2nd Half FY2025: 1-year clinical data is expected
JR-446	MPS IIIB (Sanfilippo syndrome type B)	Pre-clinical	• Under preparation for clinical trial • 1st Half FY2024: FPI in Phase 1/2
JR-471	Fucosidosis	Pre-clinical	• TBD

※Only high-priority projects in the clinical stage or soon to be in the clinical stage are listed in the above table.

On this slide, you see an overview of our R&D pipeline of those assets that are either in the clinic or soon to be, and with high priority.

Of course, one of our most important programs is JR-141 in the Global Phase III. Next to this asset, JR-171 is quite advanced in out-licensing discussions following the publication of the 52 weeks data of our Global Phase I/II study.

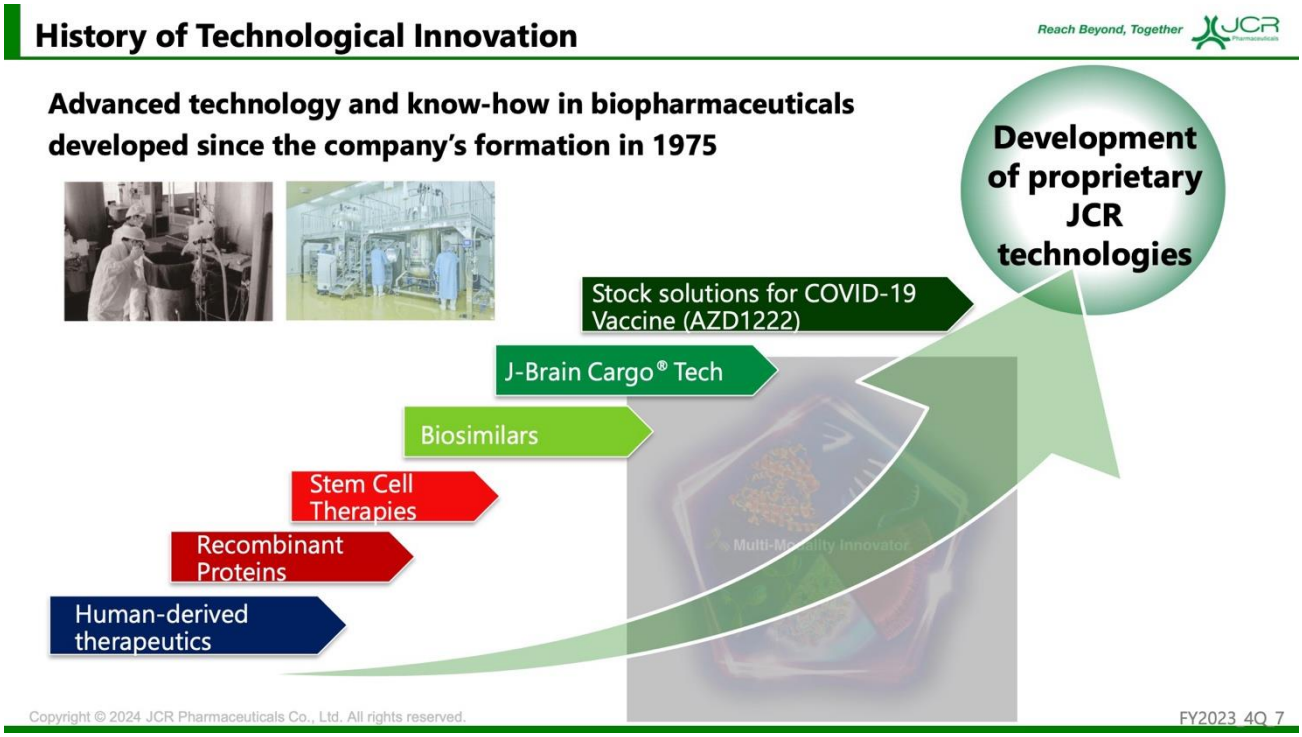
Also, the long -acting growth hormone asset known as JR-142 is on target to initiate our Phase III study in the second half of 2024, and filed for marketing authorization approximately in 2027.

Also worth mentioning here is an indication expansion trial for TEMCELL, JR-031, for the treatment of hypoxic ischemic encephalopathy in neonates.

As per the previous slide, the JR-441 Phase I/II study is progressing very well, with remarkably no hurdle in patient recruitment. Well, JR-446, in partnership with MEDIPAL, is on shuttle to start the clinical Phase I/II by the end of the third quarter of this year.

We are also working with MEDIPAL on the development of a medicine for Fucosidosis.

I will now hand over the presentation to Sonoda-san.



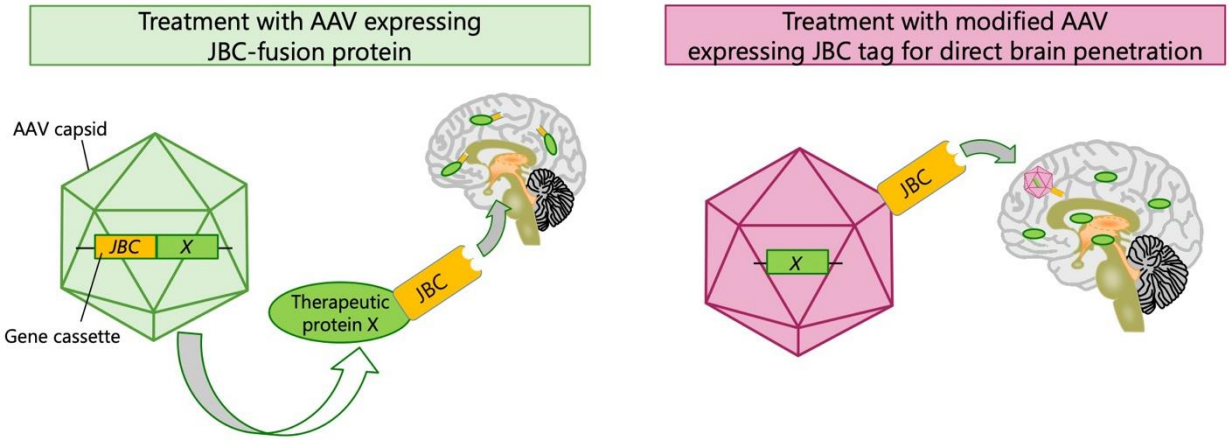
Sonoda: From here, I will talk about the next wave of technology innovation.

This slide shows the history of our technology development. Since our establishment in 1975, we have conducted research and development of biopharmaceuticals and accumulated manufacturing technologies and know-how. As a result, we have developed biosimilars and cellular medicines, with our strength in recombinant protein medicines. Based on this technology, we have also established J-Brain Cargo technology, a drug delivery technology to the brain.

In addition, we are now focusing on rare diseases and are using J-Brain Cargo technology to develop drugs for rare diseases. For areas that we do not target, or that are broader in scope, we are engaged in a variety of activities in collaboration with other companies.

Recently, gene therapy is being tried more and more in rare diseases. We have been working on gene therapy technology for many years and have been trying to develop our own gene therapy technology.

Applying J-Brain Cargo® to Gene Therapy

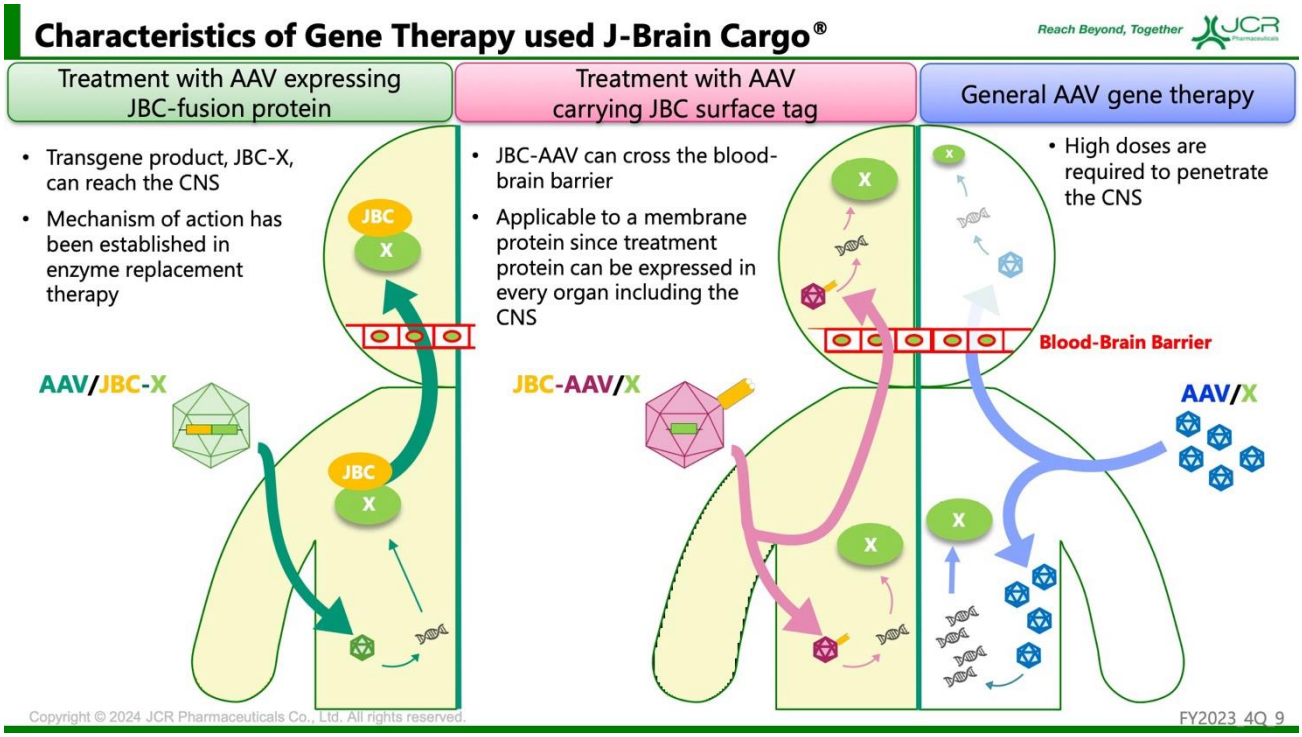


- JCR proprietary gene therapy technology applying J-Brain Cargo®
- Treatment strategies can be tailored depending on the disease characteristics

As you know, our proprietary J-Brain Cargo technology is a drug delivery technology to the brain. We have been working to establish a hybrid technology between this and adeno-associated virus (AAV)-based gene therapy.

As indicated here, we believe we have two options. The first is shown in green on the left. AAV capsid containing J-Brain Cargo and therapeutic proteins together is made and administered. After administration, J-Brain Cargo and the target protein are expressed as a fusion protein, which then reaches the brain.

The other is shown in pink on the right. This is done by attaching J-Brain Cargo molecules to the surface of the AAV itself and delivering the AAV itself to the brain. In some cases, the effects may be similar for a given disease, but one of them may be significant for a given disease. So, we believe that we have a very strong advantage in having these two types of technology.



I would like to explain in more detail. This slide shows how it is administered and what molecules are delivered to the CNS and brain after administration. As explained in the previous slide, green and pink are the two treatment methods we are developing, and the blue one on the far right is the general AAV treatment currently in use. It is shown schematically.

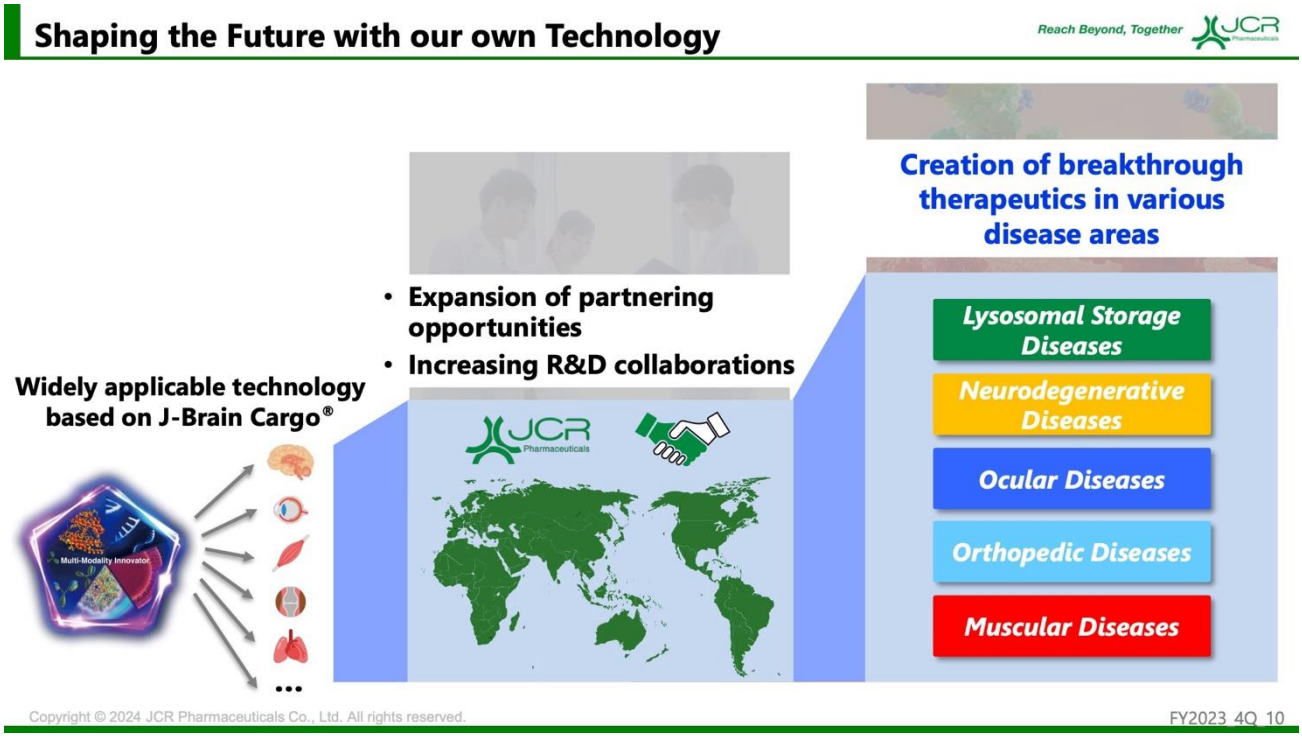
In the leftmost green treatment, as I mentioned earlier, we put a gene that is a fusion of J-Brain Cargo and protein in a common capsid and administer it. The system is such that the administered AAV primarily infects the liver, where it produces the target protein, which then reaches the brain in the bloodstream. This is like using our current enzyme replacement therapy to gene therapy.

The pink one in the middle is the other technology. A J-Brain Cargo molecule is attached to the surface of an AAV capsid. This has been technically very challenging and difficult, but so far we have succeeded well and obtained very good results.

This allows the AAV itself to be delivered to the CNS and brain after administration, where the gene can be expressed. Therefore, we believe that this technology will be applicable to diseases that could not be treated by putting in from the outside, such as membrane proteins, transporters, and other genes that would not have been effective unless they were actually expressed in the cells to be treated.

The rightmost treatment may not need to be explained. The current AAV is said to be delivered to the CNS a little, but still to a very low degree. So, as a result, it is a very difficult treatment to use, with a narrow window, because high doses are required and side effects are seen with these high doses.

Various companies are developing ways to deal with this. We believe that our technology can be a front runner in the technological development that is taking place today.



So far I have talked about our gene therapy. We have already confirmed that J-Brain Cargo is applicable to gene therapy and other modalities, including protein drugs, antibody drugs, nucleic acid drugs, and lipid nanoparticles.

In addition, J-Brain Cargo is a drug delivery technology for the brain, but we believe that using this base, delivery to other parts than the brain and the combination of this with many modalities will become a reality.

Our target is rare diseases. Some rare diseases cause symptoms in various organs other than the brain. I believe that JCR can independently develop therapy methods for rare diseases other than lysosomal disease by using multiple modalities and targeting technologies for various organs.

On the other hand, it is very difficult for us to proceed with clinical development alone for diseases with a very large number of patients. We are considering approaches to such diseases through technology licensing or joint research. This policy has not changed from the past.

Therefore, we are currently conducting research and development with a particular focus on the technology that will serve as the base of such approaches. We hope to use this technology to create breakthrough therapeutics for various diseases, both in-house and in collaboration with various companies.

That's all from me.

Consolidated Financial Results

Reach Beyond, Together JCR Pharmaceuticals

(Unit: million yen)

Consolidated	FY2022	FY2023				
	results	results	Year-on-year		Full-year forecast (Revised)	Progress rate
			Difference	Ratio		
Net sales	34,343	**42,871**	+8,528	+24.8%	45,400	**94.4%**
Cost of sales	8,886	**11,620**	+2,733	+30.8%	12,400	**93.7%**
Gross profit	25,456	**31,251**	+5,794	+22.8%	33,000	**94.7%**
Selling, general and administrative expenses	20,480	**23,719**	+3,238	+15.8%	22,500	**105.4%**
SG&A	11,678	**12,484**	+806	+6.9%	12,800	**97.5%**
R&D expenses	8,802	**11,234**	+2,431	+27.6%	9,700	**115.8%**
Operating profit	4,975	**7,531**	+2,556	+51.4%	10,500	**71.7%**
Ordinary profit	5,418	**7,264**	+1,846	+34.1%	10,000	**72.6%**
Profit attributable to owners of parent	3,772	**5,507**	+1,735	+46.0%	7,300	**75.4%**

Ito: I, Ito, will now present the consolidated financial results for the fiscal year ended March 31, 2024, and the forecast for the fiscal year ending March 31, 2025.

The first is a comparison between the term two years ago and the previous term. As Chairman Ashida mentioned, our sales were JPY42.8 billion, the highest in our history excluding the year in which we produced AstraZeneca's stock solution. Operating profit was JPY7.5 billion, with both net sales and operating profit up YoY.

Breakdown of Net Sales – Consolidated

Reach Beyond, Together JCR Pharmaceuticals

(Unit: million yen)

	FY2022	FY2023				
	results	results	Year-on-year		Full year forecast (Revised)	Progress rate
			Difference	Ratio		
GROWJECT®	12,261	**17,913**	+5,652	+46.1%	19,500	**91.9%**
IZCARGO®*	4,414	**5,171**	+757	+17.2%	5,200	**99.4%**
TEMCELL®HS Inj.	3,404	**3,236**	(168)	(4.9)%	3,300	**98.1%**
Treatments for renal anemia	4,696	**4,652**	(44)	(0.9)%	5,000	**93.0%**
Epoetin Alfa BS Inj. [JCR]	2,710	**1,994**	(716)	(26.4)%	2,200	**90.6%**
Darbepoetin Alfa BS Inj. [JCR]	1,986	**2,658**	+672	+33.8%	2,800	**94.9%**
Agalsidase Beta BS I.V. Infusion [JCR]	964	**1,661**	+697	+72.2%	1,400	**118.6%**
Total Core products	25,741	**32,636**	+6,895	+26.8%	34,400	**94.9%**
Income from contractual payment	6,546	**7,413**	+867	+13.3%	8,100	**91.5%**
Other*	123	**2,820**	+2,697	+2192.7%	2,900	**97.2%**
AZD1222 stock solution	1,931	**—**	(1,931)	(100.0)%	—	**—**
Total Net sales	34,343	**42,871**	+8,528	+24.8%	45,400	**94.4%**

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

Let's look first at the breakdown of sales.

The green area is the actual results. As you can see, we had a large increase in revenues due to GROWJECT, and IZCARGO also performed well.

Another area that was unique in this period was the “Other”, which is listed two rows below “the total core products”. This has significantly increased by JPY2,697 million over the previous year. As I mentioned previously, contract manufacturing projects contributed significantly to the increase in sales, resulting in an overall sales increase of JPY8.5 billion.

Please return to the previous slide. What I just mentioned is sales. This large increase in sales offset the JPY2.4 billion increase in R&D expenses over the previous year, and operating profit increased JPY2.5 billion from the previous year to JPY7.5 billion.

On the other hand, both sales and operating profit unfortunately did not reach the full-year forecast. As for the breakdown, I will begin with sales.

GROWJECT sales were JPY1.6 billion short of the forecast. In addition, contract revenue, shown below, was JPY0.7 billion less than planned. Overall, sales were JPY2.5 billion below the plan.

On the other hand, among SG&A expenses, R&D expenses exceeded the forecast of JPY9.7 billion by JPY1.5 billion. Together with the decline in sales mentioned earlier, overall operating profit was less than approximately JPY3 billion below the forecast.

These are the results for the previous fiscal year.

Consolidated Financial Results FY2024 (Forecast)

Reach Beyond, Together JCR Pharmaceuticals

(Unit: million yen)

Consolidated	FY2023	FY2024(forecast)		
	results	forecast	Year-on-year	
			Difference	Ratio
Net sales	42,871	**41,300**	(1,571)	(3.7)%
Cost of sales	11,620	**10,400**	(1,220)	(10.5)%
Gross profit	31,251	**30,900**	(351)	(1.1)%
Selling, general and administrative expenses	23,719	**25,500**	+1,781	+7.5%
SG&A	12,484	**12,500**	+16	+ 0.1%
R&D expenses	11,234	**13,000**	+1,766	+ 15.7%
Operating profit	7,531	**5,400**	(2,131)	(28.3)%
Ordinary profit	7,264	**4,600**	(2,664)	(36.7)%
Profit attributable to owners of parent	5,507	**3,700**	(1,807)	(32.8)%

Next, I would like to explain our forecast for the fiscal year ending March 31, 2025. The sales forecast is JPY41.3 billion and the operating profit forecast is JPY5.4 billion; both are expected to be slightly negative compared to the previous fiscal year.

Breakdown of Net Sales – Consolidated FY2024 (Forecast)

Reach Beyond, Together JCR Pharmaceuticals

(Unit: million yen)

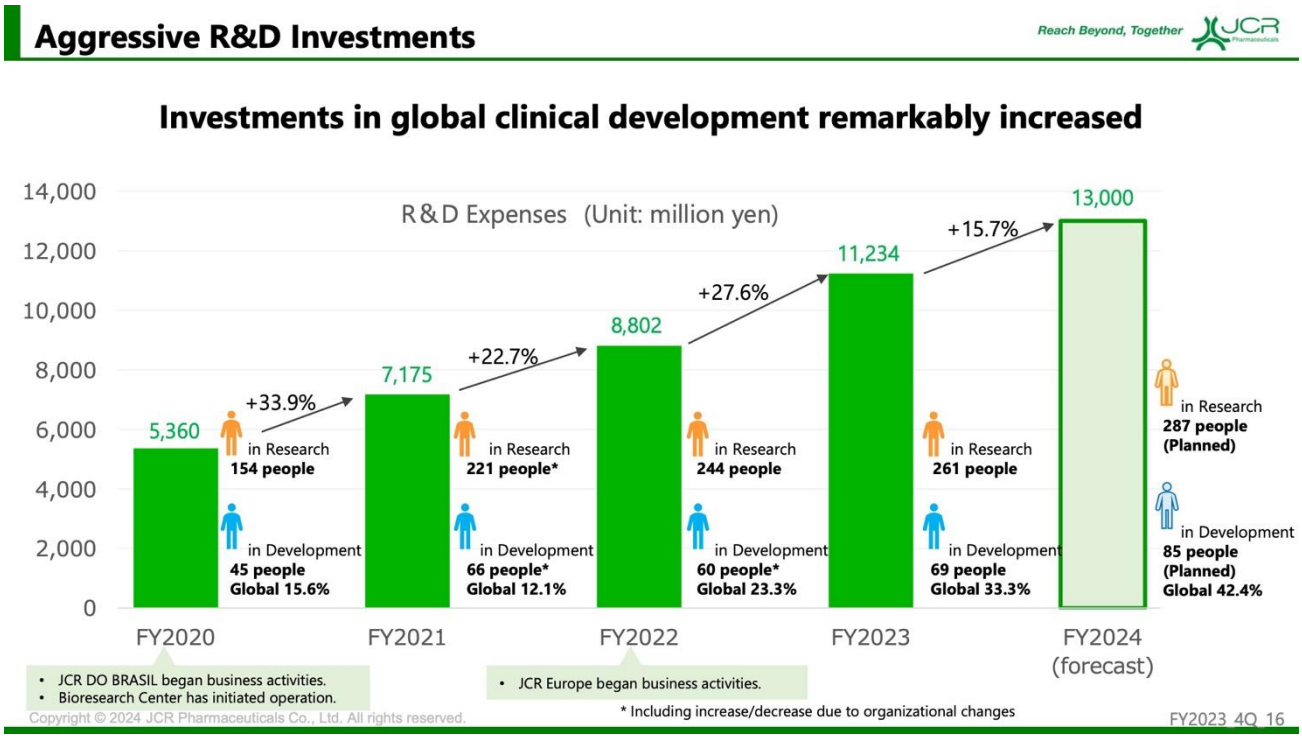
	FY2023	FY2024(forecast)		
	results	forecast	Year-on-year	
			Difference	Ratio
GROWJECT®	17,913	**18,300**	+387	+2.2%
IZCARGO®*	5,171	**5,700**	+529	+10.2%
TEMCELL®HS Inj.	3,236	**2,800**	(436)	(13.5)%
Treatments for renal anemia	4,652	**4,200**	(452)	(9.7)%
Epoetin Alfa BS Inj. [JCR]	1,994	**2,200**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

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

First, let's look at the breakdown of sales.

I mentioned that sales will decrease by JPY1.5 billion from the previous fiscal year, and the biggest reason for this decrease is found in the “Other” at the bottom. In the current fiscal year, contract production which existed in the previous fiscal year is largely negative and will result in a large negative impact of JPY1.7 billion.

On the other hand, looking at products, sales are expected to grow for both GROWJECT and IZCARGO. Income from contractual payment is also expected to increase slightly from the previous fiscal year. In addition to the decrease in sales just mentioned, R&D expenses are expected to increase by JPY1.7 billion to JPY13 billion, and operating profit is expected to be JPY5.4 billion.



As Chairman Ashida mentioned at the beginning of this presentation, we are going to invest aggressively in research and development in particular. This slide shows the trend.

The overall R&D expenditure for FY2020 was JPY5.36 billion. In comparison, we are projecting JPY13 billion for the current fiscal year and a large increase in personnel as shown here. Characteristically, research expenses had been much higher than development expenses among R&D expenses until FY2020, but from around FY2022 onward these expenses are almost equal to each other. In the current fiscal year, development expenses are expected to exceed research expenses.

Sorry, I forgot to mention one thing. Please go back to the slide before the previous one. In this slide, I mentioned that operating profit will decrease due to a large increase in R&D expenses. However, if we add back the R&D expenses and look at operating profit before R&D expenses deduction, it will be JPY13 billion plus JPY5.4 billion for the fiscal year ending March 31, 2025, which is JPY18.4 billion. The figure for the previous fiscal year was JPY18.8 billion, and so, on a basis excluding R&D expenses, there was not much change in performance.

That is all from me. Thank you very much.

Moderator: Thank you for your attention.

Question & Answer

Moderator [M]: We will now move to the question-and-answer session. The question method is shown on the screen. When it is your turn to ask a question, I will call your name. Please unmute and mention your company name and your name before asking your question. Please note that questions will be asked in a question-and-answer manner, with each person limited to two questions at a time, but you may raise your hand as many times as you like. We will now begin the session. First of all, Mr. Yamaguchi, please ask your question.

Yamaguchi [Q]: I am Yamaguchi from Citi. Thank you very much. First, on page nine, you introduced two new J-Brain Cargo technologies. In particular, you said that this middle one, treatment with AAVs carrying JBC surface tag, is new and difficult. Did you mean to say that you are going to focus on a full-scale brain treatment pipeline using this technology in red, rather than in green?

Since the treatment on the left is ordinary therapy, I would like to know about the impact and attention level of the treatment described in red.

Sonoda [A]: I, Sonoda, would like to answer your question. As Mr. Yamaguchi just mentioned, this middle treatment will target diseases that could not be treated with enzyme replacement therapy in the past, and I believe that the range of indications would be very broad.

Enzyme replacement therapy, as the name implies, replenishes enzymes, which was possible because of lysosomal diseases. Simply replacing this with gene therapy is probably not enough for us and for everyone around us.

With the therapeutic technology called AAV in the middle, any gene can be administered to the brain, so we would like to actively use it for diseases that are not lysosomal diseases, and we would like to use it more and more in the development of treatments for such diseases.

We will inevitably focus on rare diseases, but in other areas, we would like to collaborate with other companies to successfully expand this technology.

Yamaguchi [Q]: Thank you. Is it theoretically possible to have a red-colored treatment for Parkinson's disease, for example?

Sonoda [A]: Yes. However, in the case of Parkinson's, the site to be targeted is inevitably limited. Whether it is better to use a localized shot or one that spreads to the CNS (central nervous system) in general will depend largely on which target is being treated and with what. However, the answer is yes.

Yamaguchi [Q]: Thank you. Is it difficult to target Alzheimer's with this treatment?

Sonoda [A]: I think it depends on what molecules you put for Alzheimer's disease as well. However, it is generally conceivable that a molecule that binds Aβ could be administered to the brain in this way, but as you know, Aβ antibodies cause certain adverse reactions, so it will be necessary to verify whether such a molecule would be suitable for this gene therapy.

Yamaguchi [Q]: I understand. Thank you very much. Second, you showed us various charts and other information about individual sales of growth hormones for the current fiscal year. I think that although it grew quite a bit in the last fiscal year, at the end it fell a little short of what you had anticipated. You forecast an increase in sales in the current fiscal year, but is there any risk of not reaching the target? I know this is a story

of the other company, but could you please explain a little more about the situation with Novo Nordisk Pharma?

Toru Ashida [A]: I am Ashida. Thank you for your question. We believe that the main reason for the slightly lower than budgeted sales of GROWJECT in the last fiscal year was that Novo Nordisk Pharma resumed supply several months earlier than we had anticipated.

Sales for the current fiscal year are planned to exceed those of the previous year. As I mentioned at the briefing last November, we have acquired more than 200 new medical accounts in the last fiscal year. First, we are to increase the rate of new patients.

In addition, in the previous fiscal year, the affect of Novo Nordisk's supply limitation did not really show up in the figures until June. The sales volume itself has been maintained in the current fiscal year and we will benefit from this throughout the year. Therefore, we expect to be able to achieve this target and are currently working on it.

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

Moderator [M]: Thank you very much. Next, Mr. Hashiguchi, please ask your question.

Hashiguchi [Q]: My name is Hashiguchi from Daiwa Securities. Thank you. First, in your opening remarks, President Ashida mentioned that you are strengthening your in-house development capability. In your explanation on the slide, I think you also said that JR-171 is under negotiation for out-licensing. What kind of pipeline do you plan to develop in-house globally, and what kind of pipeline do you plan to develop in partnership with other companies?

I understand that your company's policy has been shaky to some extent in the past. I understood that, in the recent past, the Company was relatively inclined to seek help from other companies for items with a large market, such as JR-141. I feel that the scope of what you want to do in-house is becoming broader again. Could you please unveil a little more of your thought?

Shin Ashida [A]: We are trying to figure out how to create a clinical development system. Finally, in the middle of last year, we began to understand how we should work on in-house clinical development. We have a head office for global clinical trials in the Netherlands in Europe and three other locations in the United States, Japan, and Brazil, which are sufficient for our current clinical development for lysosomal diseases.

However, whether or not we can sell them is very problematic. So, we will consider putting out those that need a strong sales network while negotiating with the other party.

Hashiguchi [Q]: Thank you very much. The second point is about the future development of JR-171. At the last briefing, I believe you explained that a Phase III study would be commenced in FY2024, but this time you explained that an extension study is underway. Does this suggest that the data available so far does not yet allow you to enter a Phase III study?

Shin Ashida [M]: Anne-san, could you please?

Bechet [A]*: The data that we have collected on the Phase I/II together with the Extension study would be supportive for the start of the Phase III study. However, we are in partnering discussions and the Extension study at present is not fully completed yet, so we need to have both those points completed before we start the next phase of development of JR-171.

Hashiguchi [Q]: Since that is the situation, it is becoming difficult to specify the timing.

Ito [A]: Thank you for your question, Mr. Hashiguchi. We are currently negotiating the out-licensing of JR-171, and we are determined to make this a success.

For this Phase III, we believe that how to do this will be decided through discussions with the out-licensee. If we are able to do this through a licensing partner rather than proceeding on our own, this will allow us to concentrate and accelerate our own resources on the JR-141, JR-441, and JR-446 pipelines, thereby shortening the time to market as much as possible. This is what we are thinking about and focusing on.

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

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

Sakai [Q]: My name is Sakai from UBS Securities Japan. First, I am a little concerned that the plan was not met with a slowdown, partly because considerable costs were used in Q4 of the term that ended. Now that you mentioned about GROWJECT, I somehow felt that your business was not in control in Q4, including the use of expenses. Can you assure us that we need not be apprehensive about it this fiscal year?

Ito [A]: I, Ito, will answer. Thank you for your question, Mr. Sakai. Regarding the sense of slowdown in Q4 especially with regard to sales, I think it overlaps with our earlier answer about GROWJECT.

As for expenses, we believe that we were still able to record a reasonable profit despite the increase in R&D expenses throughout the fiscal year, as I mentioned earlier. We do not feel that we slowed down or used too much expenses in Q4.

From that standpoint, we will continue to increase sales this fiscal year, especially for our products. Also, regarding research and development, especially development, we will proceed as we envision. Although this will increase costs, development will proceed with the establishment of a system. By doing so, I repeat, we would like to proceed as close as possible to bringing the product to market.

As a result, we expect a decrease in profit. However, as I mentioned earlier, if we consider operating profit before deducting R&D expenses, there is not much change between last year and this fiscal year. We consider it very important that we are now in a situation where we can invest funds in such research and development.

Sakai [Q]: I understand. Now you also mentioned the plan for this fiscal year for R&D expenses. I would like to ask you about how you make a forecast. First, I would like to ask about the use of R&D funds. This year, you are talking about increasing this cost considerably. The chairman also said that development costs will increase more than research costs.

Maybe for JR-141 or JR-142, but I would like to know if there are any projects that you put a special emphasis on and invest development money in.

Regarding JR-171, I also got the impression that negotiations are in progress or will be in full swing after the current Phase I and Phase II studies are completed. Ultimately, then, I think that means that you are not expecting JR-171 license-out fees or upfront fees at this time in income from contractual payment for this fiscal year. Can you please tell me if this understanding is correct?

Shin Ashida [A]: In terms of development, we would like to focus on JR-141, JR-441, JR-142, and JR-446. We expect to be able to license out the JR-171 in a not too late period. We do not believe that this will take one or two years.

Sakai [Q]: So, it would not take a year or two, but I understand that you did not include it in the forecast for this fiscal year, is that correct?

Ito [A]: Mr. Sakai, this is Ito. You ask if the income from contractual payment includes that for the JR-171 licensing-out. Income from contractual payment for the current fiscal year includes the out-licensing of JR-171. However, we have not factored in the full amount of what we expect but made rather a conservative estimate.

As Chairman Ashida mentioned, this is a proof that we think it will not take too much time to license out it.

Sakai [Q]: Sorry for being so persistent. If so, do you expect that amount that corresponds the amount of increase in the current fiscal year?

Ito [A]: I cannot say how much money we expect to receive. We appreciate your understanding.

Sakai [M]: I understand. I will ask again later if there is anything else. Thank you very much.

Moderator [M]: Thank you very much. Next, Mr. Muraoka, please ask your question.

Muraoka [Q]: Hello. I am Muraoka from Morgan Stanley. Thank you. As a continuation of what you just said, I understand that there is some JR-171 up-front payment in income from contractual payment for this fiscal year. Is the JPY13 billion R&D expense for this fiscal year planned based on the assumption that the burden of JR-171 will be considerably reduced and that the burden of Phase III will be almost zero? Is the budget prepared on the assumption that partnering will reduce the burden by how much? Also, if the partnering at JR-171 is successfully finalized, do you think there is a possibility that R&D expenses will be lower than JPY13 billion next fiscal year?

Ito [A]: Thank you for your question, Mr. Muraoka. Regarding R&D expenses for this fiscal year and JR-171 development expenses, we have set our development expenses based on the situation I mentioned earlier.

What the figures will be for the next fiscal year and beyond will naturally depend on the progress of other items, even if we assume that out-licensing has been decided. So, I would like to refrain from saying how that will be at this time.

Muraoka [Q]: Thank you very much. But having said that, perhaps the second largest amount of development costs for your company right now, after JR-141, is JR-171. If this is reduced considerably, I would guess that the impact of R&D expenses on profit will be considerably lessened from the next fiscal year onward.

Shin Ashida [A]: It certainly reduces the development costs of the JR-171, but there are other things that could go into clinical development besides the ones that were on the table that Anne Bechet described. They will cost the same as well. Looking to the future, how much we spend on clinical development and research will have a very large impact on the future growth of our company five to six years from now. Given this, I believe that we need to invest now in the future as much as we can, to the extent that we can. We would very much like to make such investments while we can and prepare for the future.

Muraoka [Q]: I understand. Thank you very much. The other point is about the AAV with J-Brain Cargo attached, which Mr. Sonoda explained about. Sorry, I missed that. Was the application to AAV that Takeda Pharmaceutical stopped doing the green one or the red one? If it was the red one, the treatment with AAV carrying JBC surface tag that your company wants to do, what is the IP or technology transfer aspect of it?

Sonoda [A]: Now, it is difficult to answer which it was, but there is no problem regarding IPs, etc. JCR holds all IP, rights, and such. Those for both green and red are held by JCR.

Muraoka [Q]: Understood. So, your company is free to develop and there are no restrictions whatsoever?

Sonoda [A]: You are right. We can do this on our own, or we can partner with new partners.

Muraoka [Q]: I understand. How many years would it be before the red one enters clinical practice?

Sonoda [A]: We can now make investigational drugs with proteins. We have experience in protein and cellular medicine, as well as contract manufacturing of AstraZeneca's COVID-19 vaccine. We have experience with viral vectors, of course, but that was adenovirus vectors.

If the adeno-associated virus vector will be used to create an investigational drug, so it is difficult to give a definite number of years here, but I think two to three years is a general answer.

Muraoka [Q]: Thank you very much. Since you only mentioned manufacturing, does that mean that you have already made a very good product and all you have to do is manufacture it and send it down for clinical trials?

Sonoda [A]: The other is the target disease. To some extent, we have focused on diseases and are conducting validation studies using model mice. However, before going to clinical trials, we need to select a disease after accumulating many things, such as whether we can really do the clinical trials ourselves, and even if we can do the clinical trials, whether we really have enough data, scoring, and findings in the disease to get approval, and whether there is a registry. That is exactly what we are doing now.

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

Moderator [M]: Thank you very much. Next, Mr. Tsuzuki, please ask your question.

Tsuzuki [Q]: My name is Tsuzuki from Mizuho Securities. Thank you very much. Regarding JR-141, it is not listed in the pipeline of Takeda's earnings presentation material, and I believe that means it is under discussion. I would be happy to receive comments on what details are being discussed, to the extent possible.

Ito [A]: Thank you for your question, Mr. Tsuzuki. We can only say that we are in discussions regarding the partnership and the program. We apologize.

Tsuzuki [Q]: I'm a little concerned, if the partnership with Takeda ends, the competing drug company, for example, is talking in the briefing about seeking expedited approval for heparan sulfate concentration in CSF (cerebrospinal fluid). Will your company be able to do so without inconvenience without Takeda? In terms of structure, are you in a position to strengthen your overseas personnel as well? This is still tentative, but I would be glad if you could answer these questions.

Shin Ashida [M]: Anne-san, could you please?

Bechet [A]*: Our degree of confidence that we can proceed without Takeda's resources and expertise is very high, and we will proceed with the program with no impact on our timelines, no matter what scenario we look forward with or without Takeda.

Tsuzuki [Q]: I understand. If there is no longer a joint development with Takeda, after the application is submitted and approved, would you look for a partner who can market the product, as you mentioned earlier?

Ito [A]: I don't think it is appropriate to answer such hypothetical questions now.

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

Moderator [M]: Thank you very much. The next question will be the last. Mr. Mizuno, please ask your question.

Mizuno [Q]: I am Mizuno of Tokio Marine Asset Management. Thank you very much today. This overlaps with Mr. Tsuzuki's current question: what is your company's position on the discussion that Denali Therapeutics Inc. is having with the FDA about the possibility of expedited approval based on the level of heparan sulfate? Can your company similarly challenge the application and expedited approval once the Cohort B data, for example, is ready? Or do you think that is just Denali's own opinion and that it will be difficult? What are your thoughts on this matter?

Shin Ashida [M]: Anne-san, could you please?

Bechet [A]*: So the FDA, of course, is having workshop concerning the potential of biomarkers in support of approval. There is a very big difference, however, between having workshops and supporting biomarkers exclusively for approval. We are having our regulatory engagement with regulatory bodies of our own and follow our plan as previously anticipated, and our own schedule for this, which should keep us in a competitive position compared to Denali.

For our part, we are discussing engagement with the authorities. And we are thinking of doing it with the plan we mentioned earlier. And I believe this will allow us to defend a very competitive and different position from Denali.

Mizuno [Q]: Regarding the gene therapy described by Mr. Sonoda, I am very aware that we cannot make the same comparison because the modalities are completely different, but I would like to know your opinion about how the ex vivo type of lentivirus gene therapy works on the brain. Also, I am sure that your system is far superior in terms of the delivery to the brain, but please tell us about the competitive environment. Can you comment on what you think is the position of another type of gene therapy than the blue one on the far right?

Sonoda [A]: Thank you very much. I think the question would be with Orchard in mind. In addition to the data Orchard is putting out, I believe there are several other data available, including the non-clinical and clinical effects of Ex vivo gene therapy on the CNS.

Looking at that data, we can never say that it is not working for CNS. We recognize that it has a certain effect on CNS. However, ex vivo may be able to cover rare diseases with extremely a few patients, but manufacturing and distribution will inevitably be a major bottleneck, and I think this is the general consensus.

Therefore, I still believe that AAV will continue to be the mainstream. As I mentioned earlier, I believe that for other diseases than lysosomal diseases, a therapy in which the AAV itself reaches the brain will have a significant advantage over ex vivo therapy.

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

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 financial results briefing for the fiscal year ended March 31, 2024, for JCR Pharmaceuticals. Thank you all very much for your participation today.

[END]

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