JCR Pharmaceuticals Co., Ltd.

Q2 Financial Results Briefing for the Fiscal Year Ending March 2023

October 26, 2022

Event Summary

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[Participants]	
[Number of Speakers]	6

Shin Ashida	Representative Director, Chairman, President and CEO
Toru Ashida	Senior Vice President, Sales and Administration Executive Director, Sales Division
Mathias Schmidt	Vice President, Clinical Development, Global Business Strategy and Business Development
Hiroyuki Sonoda	Vice President, Research and Corporate Strategy Executive Director, Research Division
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Yoshihiro Ohta	Director, Accounting Department,

		Management Division
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	Kazuaki Hashiguchi	Daiwa Securities
	Naomi Kumagai	Mitsubishi UFJ Morgan Stanley Securities
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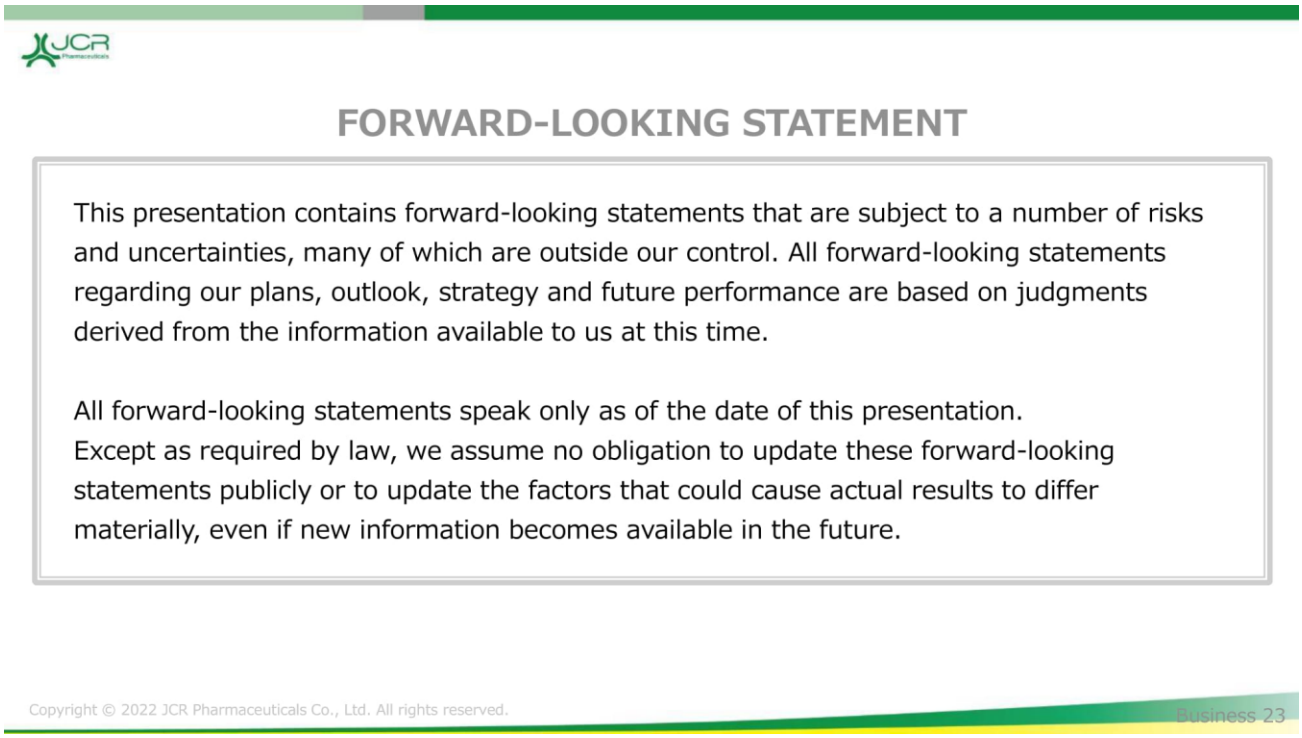
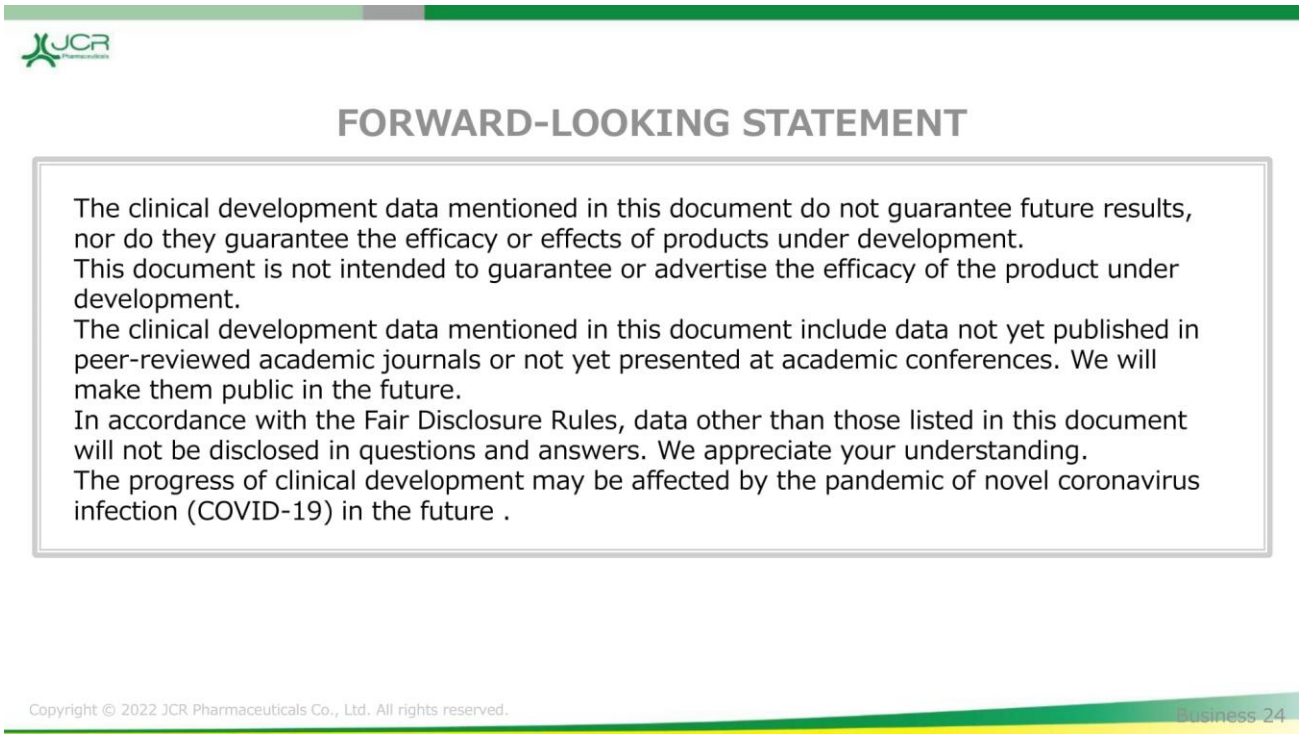
*Analysts that SCRIPTS Asia was able to identify from the audio who spoke during Q&A.

Presentation

Moderator: Now we would like to begin the 2023 H1 results briefing session.



Before I begin the briefing session, there are some housekeeping announcements to make. Some places, we are going to touch on the future forecast. They may include some risks, hypothetical statements, so for the investors, this is remark that the result or the actuals may be different from what is stated in this meeting.



Also, this is to give the general information of the situation of our company. The pharmaceutical information is not to be intended to give some medical advice or the commercial purposes.

Also, we are recording this session so that the highlight will be shared later in our homepage.

At this meeting, we ask the presenter to keep the mask. So please bear with that.

I would like to, first of all, introduce to you the presenters. To your right, Representative Director, Chairman, President and CEO of JCR Pharma, Shin Ashida; next, Senior Vice President, Sales and Administration Executive Director, Sales Division, Toru Ashida; Vice President, Clinical Development, Global Business Strategy and Business Development, Mathias Schmidt; Vice President, Research and Corporate Strategy, Executive Director, Research Division, Hiroyuki Sonoda; Senior Corporate Officer, Executive Director, Administration Division and Director, General Affairs Department, Yutaka Honda ; and then finally, Director, Accounting Department, Management Division , Yoshihiro Ota.

Further too, let me explain about the materials that we are going to cover. If you go to the homepage of JCR Pharma, you will be able to find out the presentation material. Would you please take a look at that?

Today, presentations as well as a Q&A will be held within one hour.

Mr. Ashida would like to give the opening remarks. Would you please?

Shin Ashida: Hello. This is Ashida speaking. I would like to say thank you very much wholeheartedly from the bottom of my heart for your support.

Thinking about H1 results. Compared to the last term, because of the completion of the AC injection, the business profit and also the sales came down. Contract fee. Because those contracts continue into H2 and also H1, basically, everything went along with our budget.

Now first, about the license contract. For the license contract, would you please take a look at this slide?

I would like to focus LSD, so these are the development items that we have as of today. The red highlighted ones, there are about five of them. They are called ultra-rare disease; extremely rare diseases; and very rare, with a less number of patients. Others, though, have many more numbers of patients. We classify them according to the number of patients. However, ultra-rare disease, even though the number of patients is less, the question is imposed upon us as to how we can deliver the product to them.

This touches the Company's mission, how we are going to deliver the product after going through the clinical trials. We are thinking about different schemes in order for us to do that. In the latter half of this term, we would be able to come up with clearer situation about them and others as well with more number of patients.

And so, for example, MPS Hunter Syndromes. Since last year, we've been working together with Takeda Pharmaceuticals for its development. JR-171 Hurler syndrome, for example, that is also going into the Phase I/II. Basically, the administration have been completed. So one by one, we've been collaborating with many other companies and then trying to sign on the agreements.

It's going to be tremendously hard work because each one of them, you need to do the negotiation with various companies and provide data accordingly. And so, of course, we've been doing it, but if we are able to compile them and go for the clinical trials all at once, actually, there are such companies available, and we started to negotiate with such companies.

As far as we are concerned, as much as possible, we want to go for the ultra-rare disease and also the disease with more number of patients. We are thinking about the two scheme of approaches when negotiating with the potential companies.

Also long-acting growth hormone, we have been developing that. But when it comes to the next year, we will move into the Phase III trials for that. There is a company who would like to have a license and we've been also discussing for that possibility with this company.

Also talking about J-Brain Cargo, using that, come up with the antibody and low molecular substance and use it in the brain, there are actually companies who are interested in such a scheme. We already started the negotiation with them. At the research function, of course, J-Brain Cargo is a very major thing. But the target now is the muscle. For example, step-by-step, the research has been advancing in those areas as well.

Our research and development, basic research activities, there are so many going on. For the future, would you please focus your attention to us, JCR Pharma? Thank you very much for your support in advance.

Next about the business and then financial aspects. Later, we would like to have a Q&A session.

From here, financial results will be presented, please.

Ohta: From the Accounting Department, this is Ohta to present, referring to financial results.

Please look at the first page.

As you see in the title, so with YoY, we had decreased revenue and profit, but this is as expected as we had assumed in the beginning of the year. Sales JPY15,581 million; operating income, JPY744 million; ordinary income, JPY1,569 million; and profit attributable to owners of the company, JPY962 million.

As for major products, GROWJECT due to the impact of NHI pricing revision, it had decreased revenue. However, in terms of IZCARGO, it had quite good sales, which contributed to the total sales, which is at the same level as previous year. Then the AZD1222 bulk, the contracted manufacturing was completed as well as the first half of the licensing fee income also decreased. Therefore, our sales and each profit decreased YoY.

On the other hand, R&D cost, with active R&D activities, the cost or expenses are increasing for R&D.

Please look at the next page.

In the middle, in the green part, this is the results for this term. On the right-hand side, you can see the factors for changes, vaccine sales, as well as the licensing fee revenue. The sales decreased so the impact was minus JPY12,802 million. Our R&D costs increased by JPY696 million. Therefore, that is a contributing factor for minus operating income. Specific to the time range ending in March 2022, the SG&A cost is offset by JPY954 million on the right-hand side.

In the table, you can see the full-year forecast. As we projected for H1, it is progressing. For H2 as well, there are no major changing factors. Therefore, we are keeping the forecast from the beginning of the year.

For sales, JPY45 billion and operating income of JPY14,500 million; ordinary profit, JPY14,500 million; and net profit of JPY10,300 million. This is the breakdown of sales.

As for pharmaceutical, JPY12,583 million. You can see on the right-hand side, the changes as for the factors for increase. IZCARGO, also plus-JPY1,132 million due to steady penetration. However, AZD1222 bulk, minus-JPY6,114 million, and also licensing fee revenue down JPY6, 547 million.

For H1, each product has a projected enhanced progress. Therefore, for the H2 forecast, we are keeping the original forecast. Total sales we are projecting is JPY45 billion.

Next is the sales trend for each product.

As you can see in the graph, the white part is the full-year forecast GROWJECT, TEMCELL and Agalsidase YoY, we are projecting increased revenue. And as for IZCARGO, the accumulated quarterly sales are shown here. We can see the steady progress of sales. Here, this is total sales. For 2023, term ending in March as a full year, the licensing fees are all included in here.

Let me talk about the financial positions.

As for assets, total JPY89,387 million, so this is minus-JPY7,746 million, but because of the current assets, that's minus-JPY7,870 million. And the current asset, the accounts receivable was offsetting actually JPY6,996 million. And as for the liabilities, this is also going down.

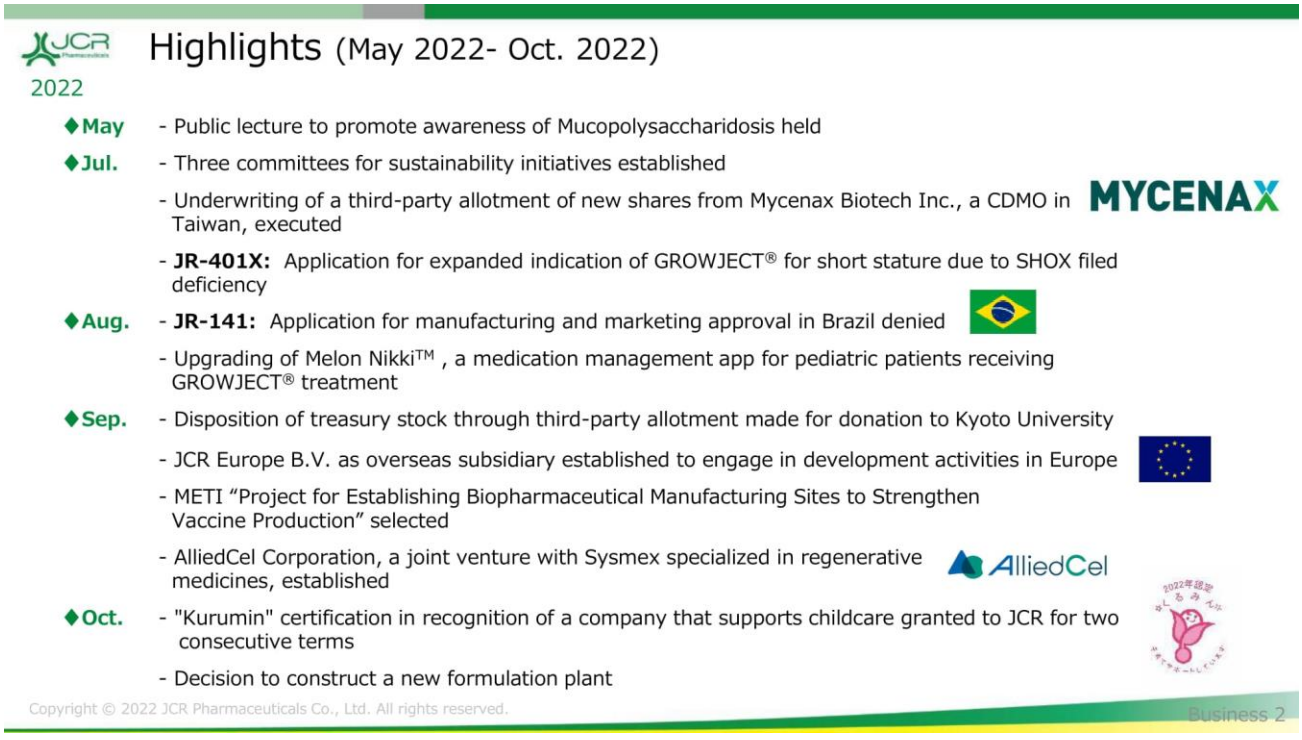
As for the cash flow, let me explain.

Cash flow from operating activities, JPY1,027 million. The major factor is that the corporate tax payment saw JPY5,516 million. Also for cash flow from investing activities, CapEx, JPY1,779 million. As for cash flow from financing activities, dividend payment was JPY1,479 million, and cash and cash equivalents was JPY28,107 million. Out of the table, as you see the depreciation, the full-year, JPY1,870 million is expected.

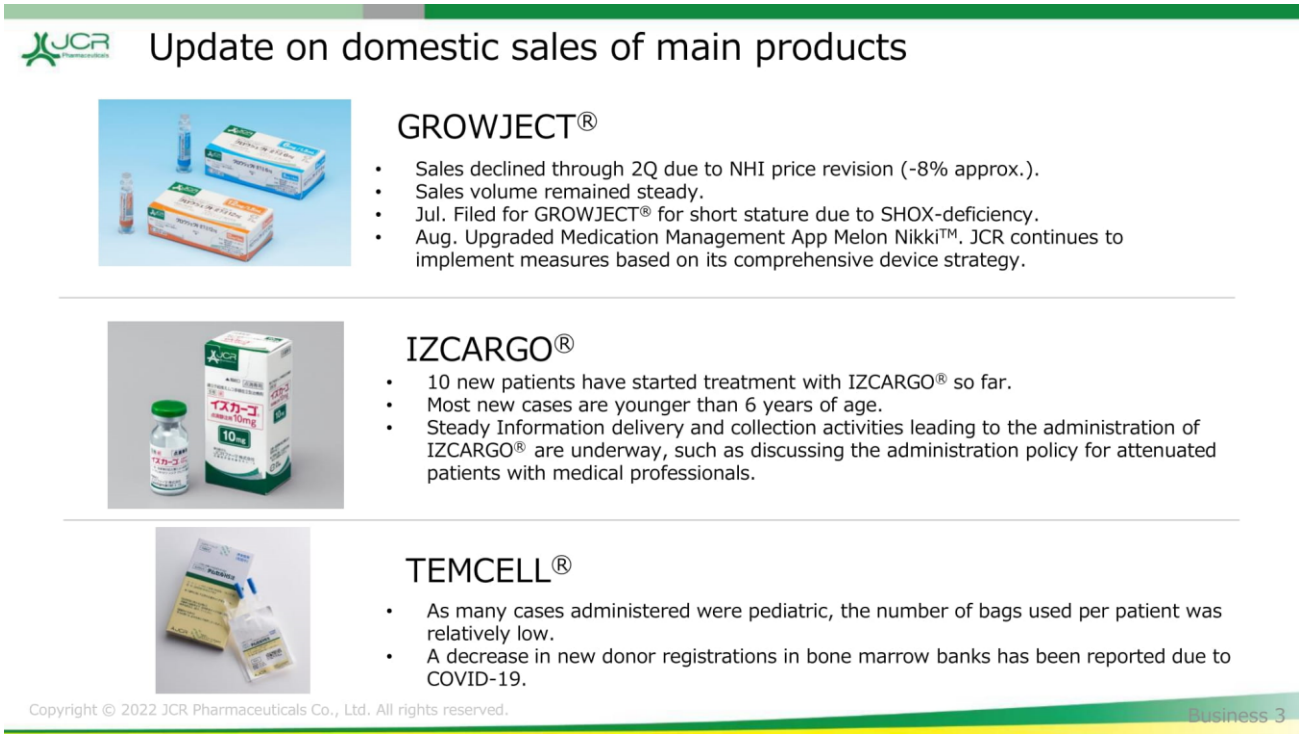
This concludes the summary of financial results. Thank you very much.

Moderator: We are continuing on to explain our businesses.

Toru Ashida: For the term ending in March 2023, let me explain. My name is Ashida and in charge of our sales division. Let me talk about the sales status in Japan as well as the sustainability activities.



This is from May 2022. These are the topics or highlights. It's been already on a press release, and I apologize, I am going to skip the detailed explanation on this.

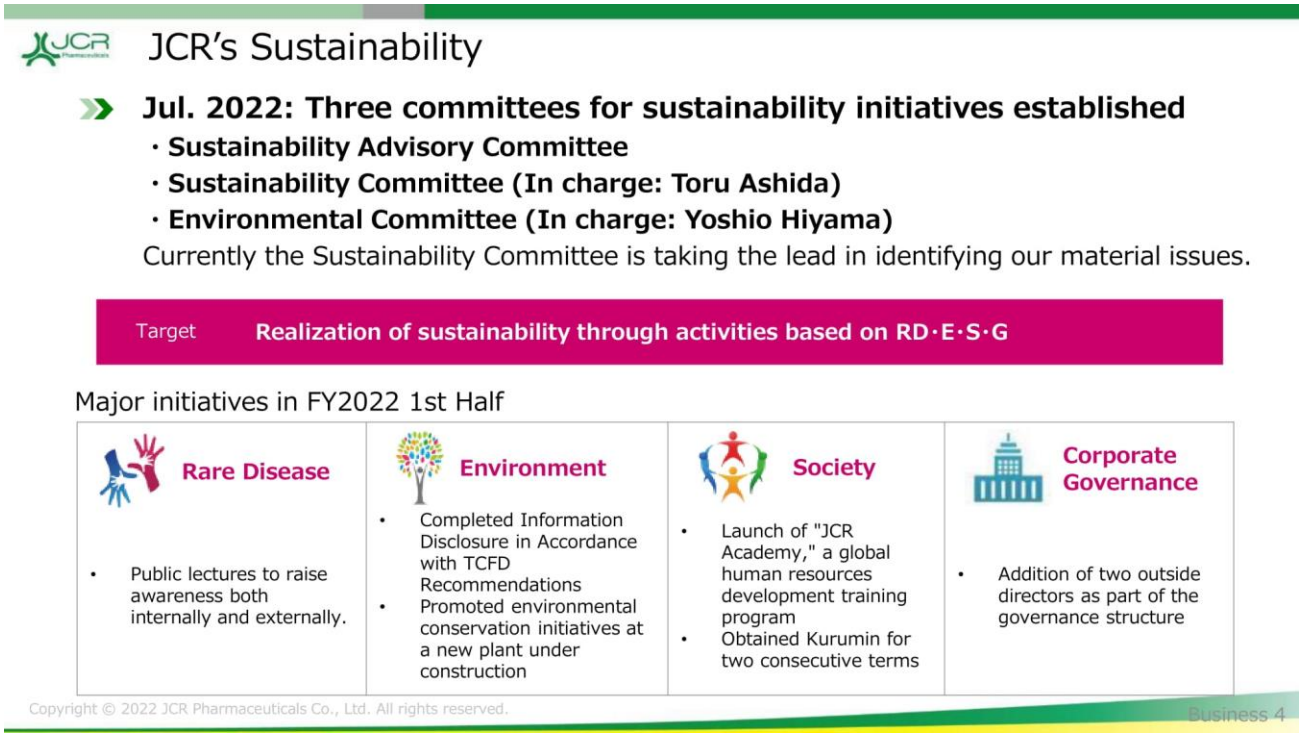


Please take a look at this slide. From here, let me update on the domestic sales of main products.

As for GROWJECT, for Q2 sales, it was down from last year. However, the sales volume remained steady. The factor of decreased sales is that minus-8% of NHI price revision. With the price revision as well as going down the birth rate, we do expect more impacts. But this July, we have filed for GROWJECT additional indication. Also in August, GROWJECTOR L, which is the only electric injector in Japan, we have upgraded the medication management app Melon Nikki. We are going to continue to implement measures based on its comprehensive device strategy to increase the number of patients.

Then next, this is the first pharmaceutical product applying J-Brain Cargo, IZCARGO. We have added 10 new patients who have started treatment with IZCARGO. Most new cases are younger than six years of age and expected efficacy was, we believe, delivered to the physicians. For attenuated patients as well, we are going to deliver more information to the special doctors, and we will make sure to have these activities focused and to more MPS II patients in Japan, we'd like to deliver IZCARGO.

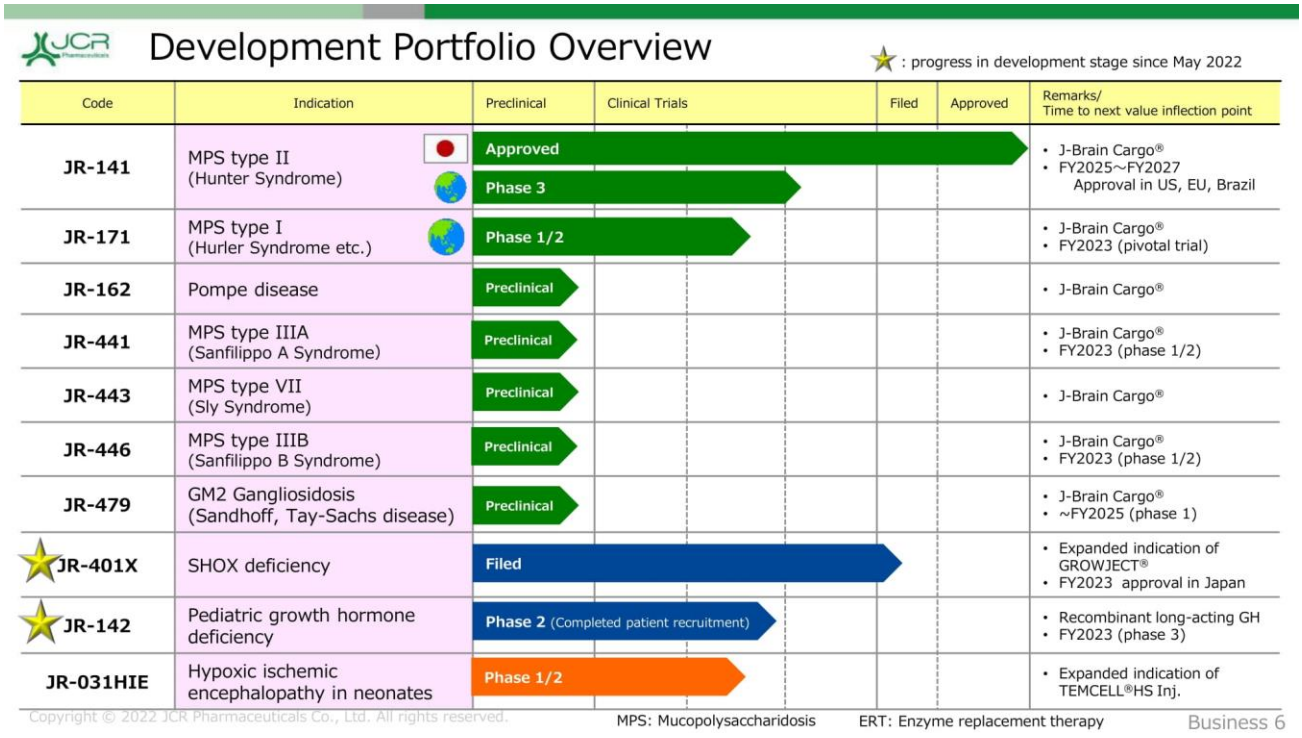
Now about TEMCELL. A number of patients and also the number of bag use per patient was increasing. I think the TEMCELL is known widely by the doctors, but TEMCELL has been increasing. But when it comes to the users, they are mostly pediatric. Per the patient, the number of bags used actually was coming down in number. And because of the COVID-19 situation, the bone marrow banks, the donor registration is declining. So we've been investigating further the situation in the market of TEMCELL right now within the Company.



Next, about JCR sustainability activities.

In July this year, we have established three committees for sustainability initiatives. We would like to realize the sustainability activity is so unique to JCR. We'd like to perfectly implement them. The Sustainability Committee that I'm in charge of, we are trying to identify the materiality. The rare disease ESG-related business activities have been in action, and we would like to accelerate these activities into the future.

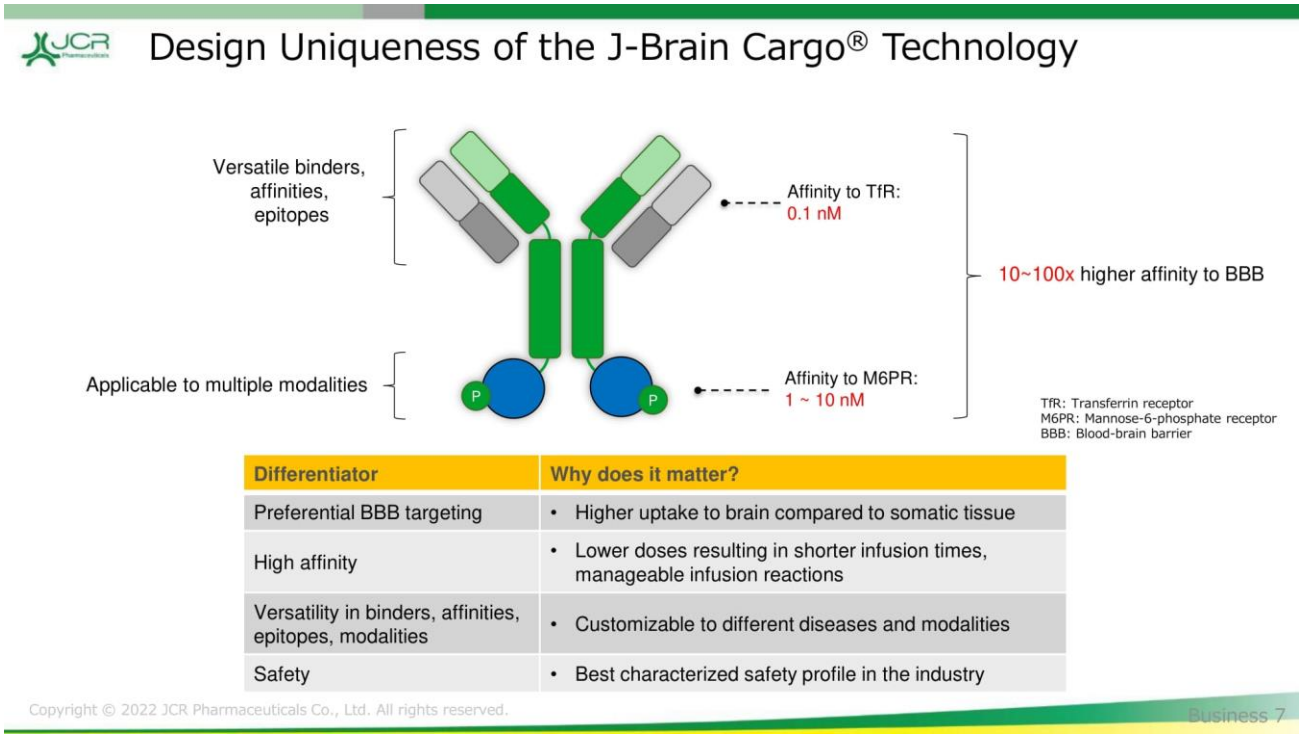
That's all from me. Thank you very much for your kind attention.



Schmidt*:On this slide, you see an overview of JCR's development portfolio of programs, either in clinical trials or entering clinical trials in the foreseeable time.

Notable updates include the filing of JR-401X for the treatment of SHOX deficiency and the completion of patient enrollment for JR-142, the long-acting growth hormone.

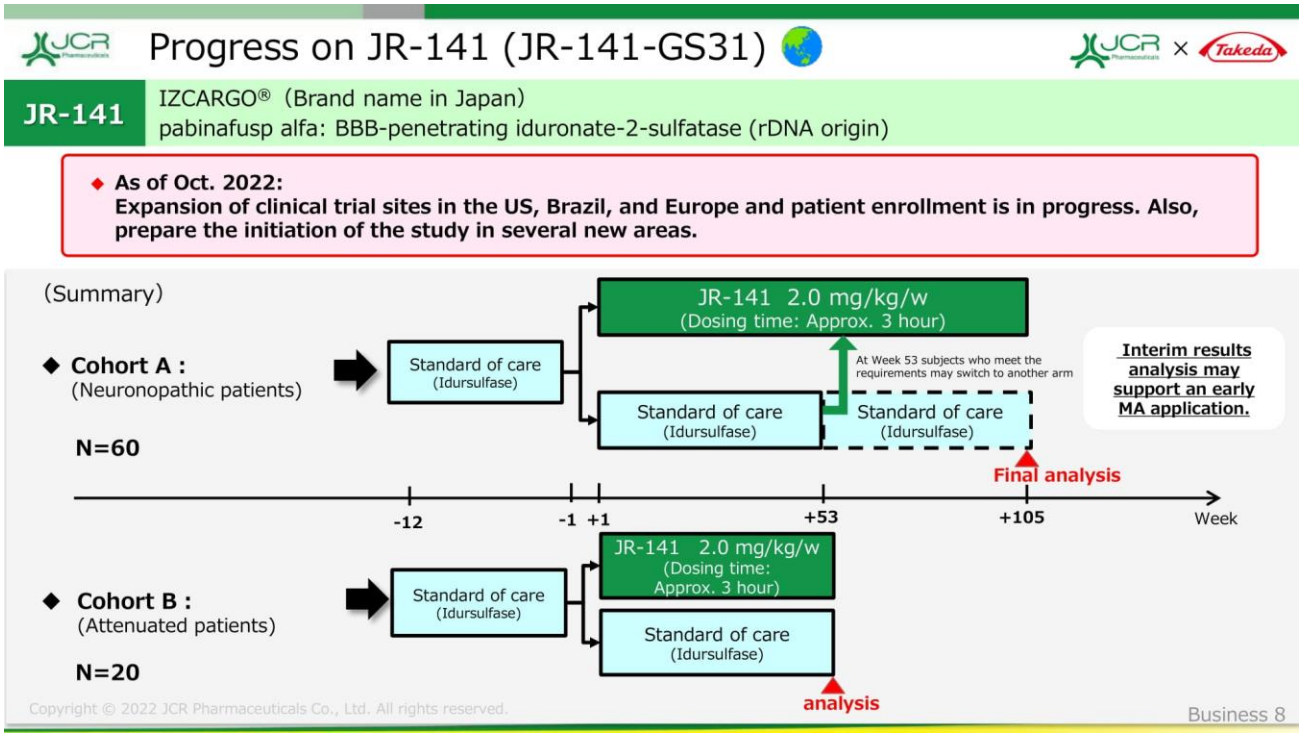
The next program is expected to enter the clinic for the treatment of MPS IIIA, which was built on the J-Brain Cargo technology, is JR-441.



We are often asked how our molecular differentiation of the J-Brain Cargo technology results in a clinical differentiation that is observed by the patients and the physician. There are three major differences.

Number one is the high affinity towards the blood brain receptor that allows the efficient delivery of the therapeutic already at low doses. As the enzyme is immunogenic and cause infusion reactions, the lower doses we can administer result in shorter infusion times. This is important for the patients. We also believe that this results in less infusion reactions.

The third point is JCR has the best established safety profile for any blood-brain barrier-penetrating technology. With multiple clinical trials successfully concluded, more than a year of marketing experience with IZCARGO and our second program based on this technology showing very favorable safety profile in the clinic.



Our global pivotal study with JR-141, or pabinafusp alfa, for the treatment of MPS II is proceeding according to plan.

We further accelerate patient recruitment so we decided to expand the countries and sites in Latin America, Europe, and the United States. Target number of subjects is 60 for Cohort A and 20 for Cohort B.

We are very happy to have Takeda as our partner and receive continued support in the conduct of the global study.

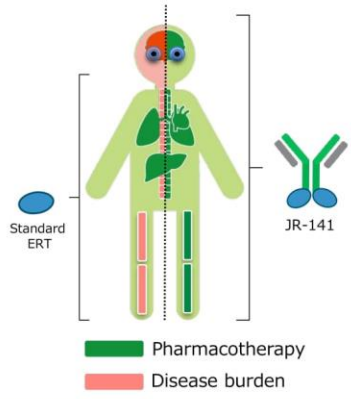
Progress on JR-141 (JR-141-GS31)

JR-141 IZCARGO® (Brand name in Japan)
pabinafusp alfa: BBB-penetrating iduronate-2-sulfatase (rDNA origin)

Objective : JR-141-GS31

1. **To demonstrate the significant efficacy of JR-141 on CNS signs and symptoms in MPS-II subjects relative to standard ERT.**
   JR-141 is expected to have superior activity on neurologic signs and symptoms of MPS-II by reducing substrate in the brain.

2. **To demonstrate control of somatic signs and symptoms by JR-141 that is comparable to standard ERT.**
   JR-141 is expected to control somatic symptoms and biomarkers comparable to standard ERT (even though some improved symptom control may be seen due to dual uptake mechanism by JR-141)

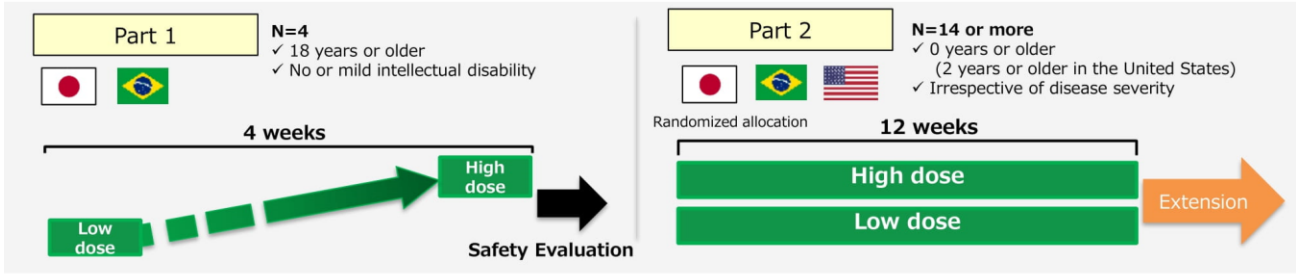


The objective of the trial is to demonstrate exactly what the platform technology is designed for: superiority of JR-141 in addressing the CNS signs and symptoms of the disease of MPS-II over standard of care and control of thematic signs and symptoms relative to standard of care.

Progress on JR-171

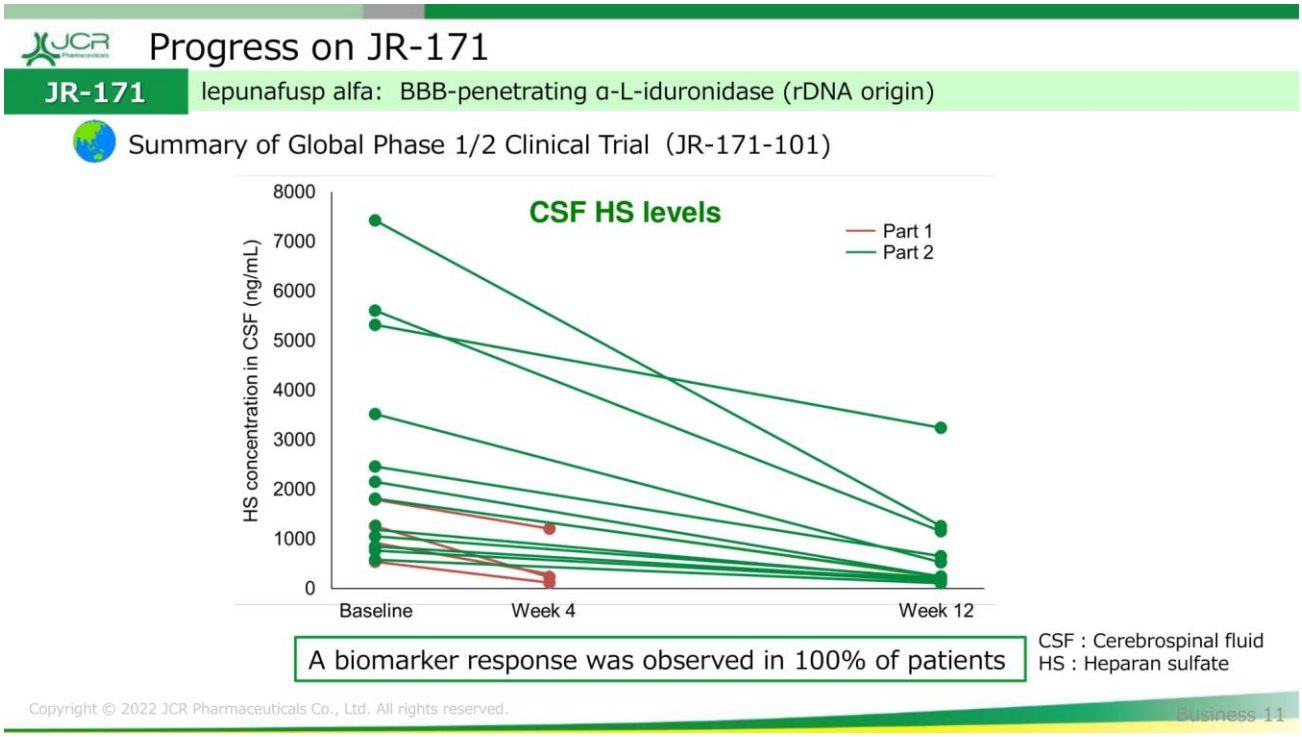
JR-171 lepunafusp alfa: BBB-penetrating α-L-iduronidase (rDNA origin)

Summary of Global Phase 1/2 Clinical Trial (JR-171-101)

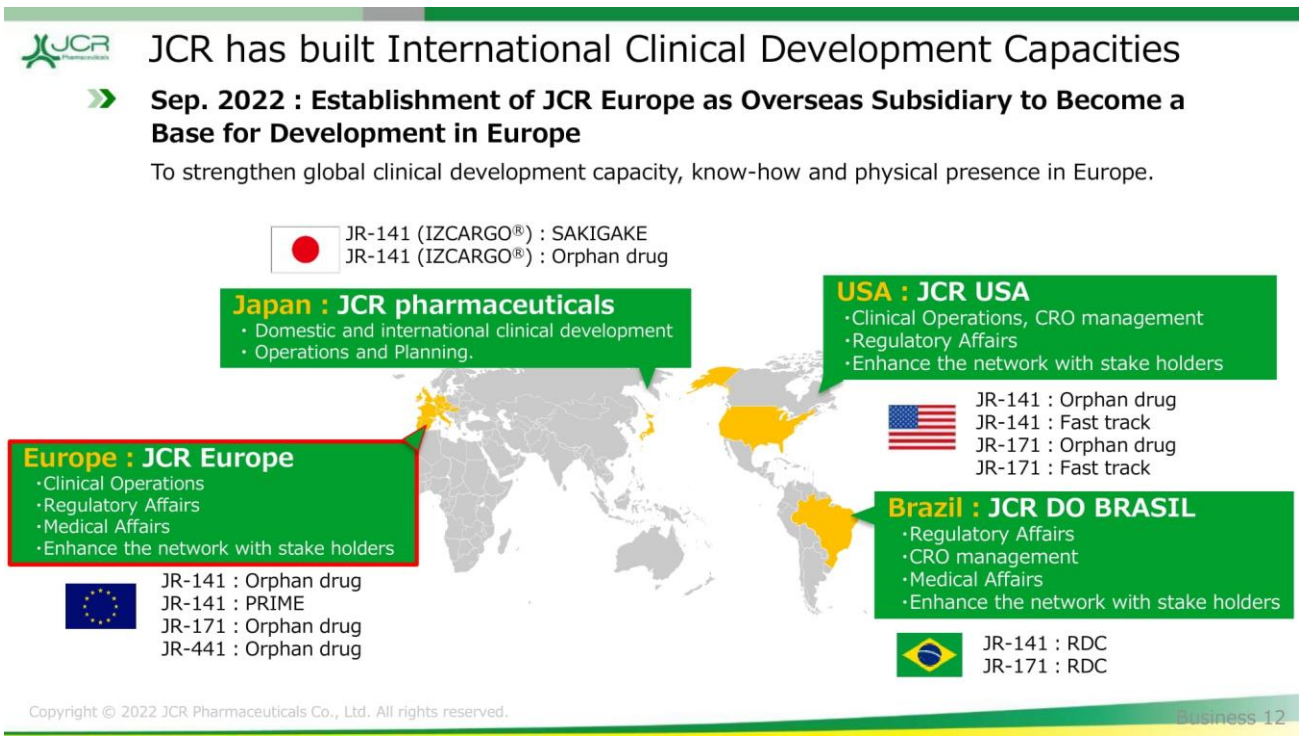


	Part 1	Part 2
Primary endpoint	Safety	
Secondary and exploratory endpoints	・Plasma drug concentration, pharmacokinetic parameters ・Exploratory efficacy for central nervous system and systemic symptoms	
Geography	Japan・Brazil	Japan・Brazil・USA
Clinical trials identifier	clinicaltrials.gov NCT04227600	

Our second asset based on the J-Brain Cargo technology that is currently in Phase I/II clinical trials is JR-171. The clinical trial is fully enrolled, and we are observing very favorable safety profile and encouraging clinical signs of efficacy. All patients are currently in Part Two of the study where we are investigating two doses.

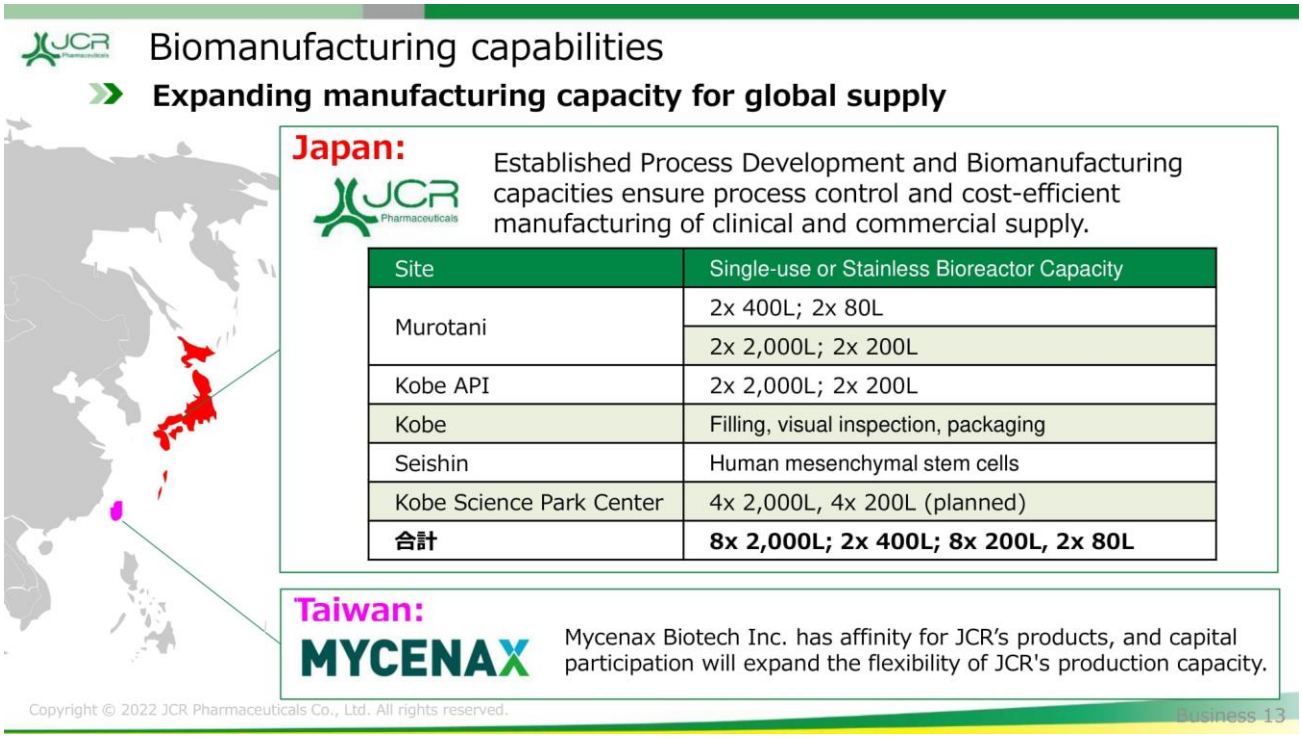


As a biomarker, we are following the reduction of heparan sulfate in the cerebrospinal fluid. This biomarker in an appropriate setting can inform the reduction of the substrate in the CNS. A quite rapid response was actually observed in all subjects enrolled, and we are very much encouraged about these data.



To enhance our ability to execute our clinical trials globally, we have expanded our capabilities in the United States and in Brazil, and we have established a new subsidiary in Europe. The new subsidiary is located in Leiden, the Netherlands, very close to the EMA and adjacent to one of the top five biotech parks in Europe.

All subsidiaries work seamlessly together in the conduct of the trials. Thereby, we ensure that we have all necessary knowledge in the geographies where we do conduct clinical trials.



With the building of the Kobe Science Park plant that was partially subsidized by a government grant, JCR has significantly increased its biomanufacturing capabilities.

In addition, we can tap into flexibly available manufacturing capacities at Mycenax. Mycenax is familiar with the JCR products so that a technology transfer process is expected to go smoothly. Here, I think we have a credible play that we can progress multiple programs in parallel into the clinic and throughout development.

Expansion of JCR's Manufacturing Facilities

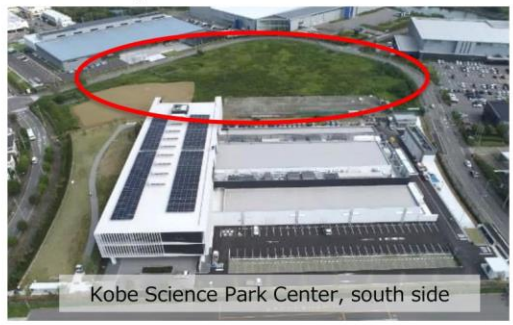
Kobe Science Park Center
(under construction)

- Utilize subsidy related to the extraordinary grant for establishment of a production system for COVID-19 vaccines
- Completion of construction: November 2022 (Scheduled)
- Start of operation: FY2023 or later


Kobe Science Park Center, west side

New formulation plant
(resolved on 10/26/2022)

- Adjacent to Kobe Science Park Center (land acquisition in March 2022)
- Utilize the Ministry of Economy, Trade and Industry's "Subsidy for Adoption of Vaccine and Other Production System Improvement Project" (subsidy amount to be determined)


Kobe Science Park Center, south side

» **Both plants will manufacture JCR's products in the absence of requests to manufacture vaccines etc.**

Importantly, we are now also expanding our fill and finish capacity by constructing a new drug product plant adjacent to our new manufacturing site at the Kobe Science Park. This plant is also partially subsidized by a government grant to ensure Japan is ready prepared for vaccine manufacturing infrastructure in case of a global pandemic.

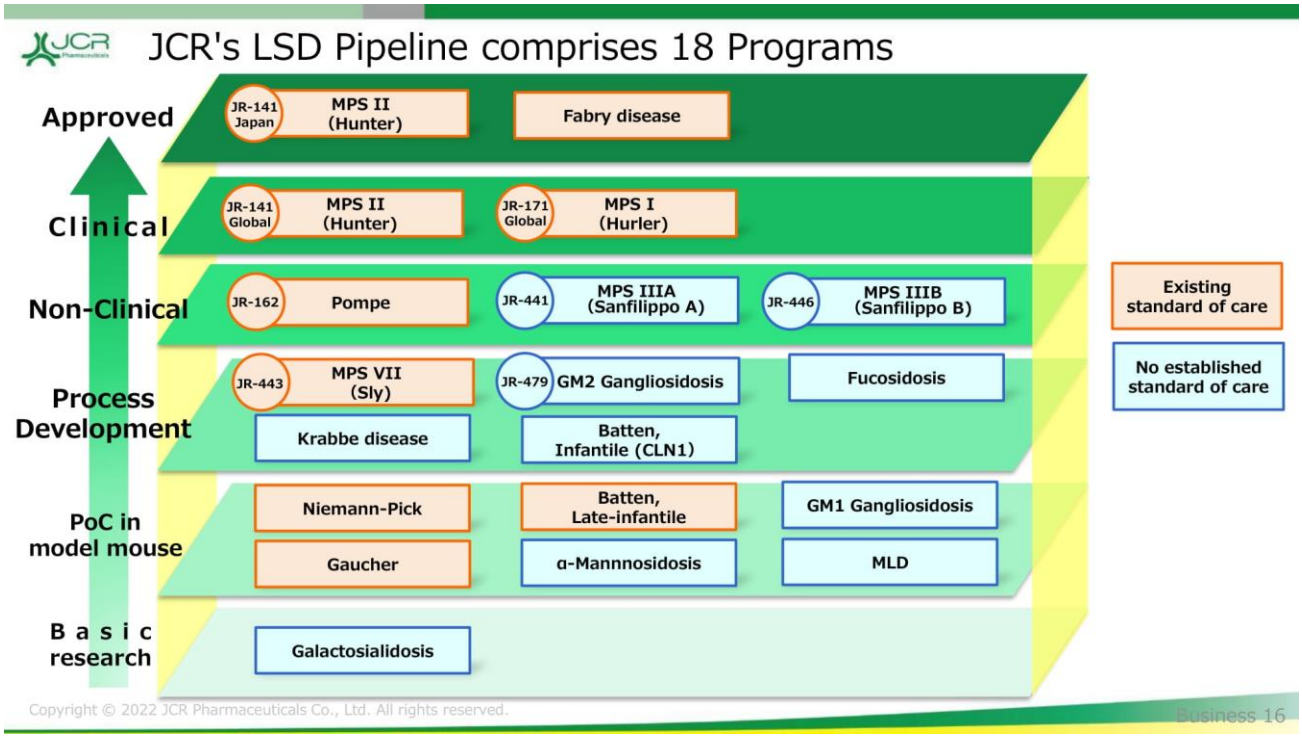
Lysosomal diseases : Expected Timelines

Note: It is a plan at this stage and is subject to change

	FY2022	FY2023	FY2024	FY2025	
JR-141 pabinafusp alfa (MPS II)	**Global : Phase 3 trial (Ongoing)**				• SAKIGAKE (PMDA) • Orphan Drug (PMDA) • Orphan Drug (FDA) • Fast Track (FDA) • Orphan Drug (EC) • PRIME (EMA)
JR-171 lepunafusp alfa (MPS I)	**Global : Phase 1/2 trial (Data analysis)**	Initiation of Phase 3 trial			• Orphan Drug (FDA) • Fast Track (FDA) • Orphan Drug (EC)
JR-441 (MPS IIIA)		Initiation of Phase 1/2 trial			• Orphan Drug (EC)
JR-162 (Pompe)	**Non-clinical (Ongoing)**				
JR-443 (MPS VII)	**Non-clinical (Ongoing)**				
JR-446 (MPS IIIB)	**Non-clinical (Ongoing)**	Initiation of Phase 1/2 trial			
JR-479 (GM2 gangliosidosis)	**Non-clinical (Ongoing)**		Initiation of Phase 1/2 trial		

Here, you see a more granular view of our near-term value inflection points of JCR's lysosomal storage disease portfolio. We are very excited to move our MPS IIIA asset, JR-441, into clinical stage mid of next year. We have received a lot of interest already from physicians and patient families. It will be followed by JR-446, a highly

differentiated asset for the treatment of MPS IIIB, and later on, by JR-479 for the treatment of GM2 gangliosidosis.



As the Chairman already pointed out, JCR has an overall portfolio of 18 programs at various stages, both in indications with no established standard of care, as well as in indications with existing therapies, which in contrast to JCR's assets do not address the unmet medical need to address the central nervous system signs and symptoms. Only the J-Brain Cargo technology can do so.

Progress on JR-142

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

Phase 2 clinical Trial (JR-142-201): Brief Summary

◆ **Completed patient recruitment** (First Patient Dosed: May 2021)

Subjects	Pediatric Growth Hormone Deficiency (Pediatric GHD)
Endpoints	• Assessment of PK/PD profile • Growth rate (after 26 weeks) • Safety etc.
Number of Subjects	24subjects
Study drug	JR-142 / GROWJECT® *Both are administered by auto-injector.
Details	jRCT(Identifier : jRCT2031200372)

Switching now to focus on JCR's growth hormone franchise.

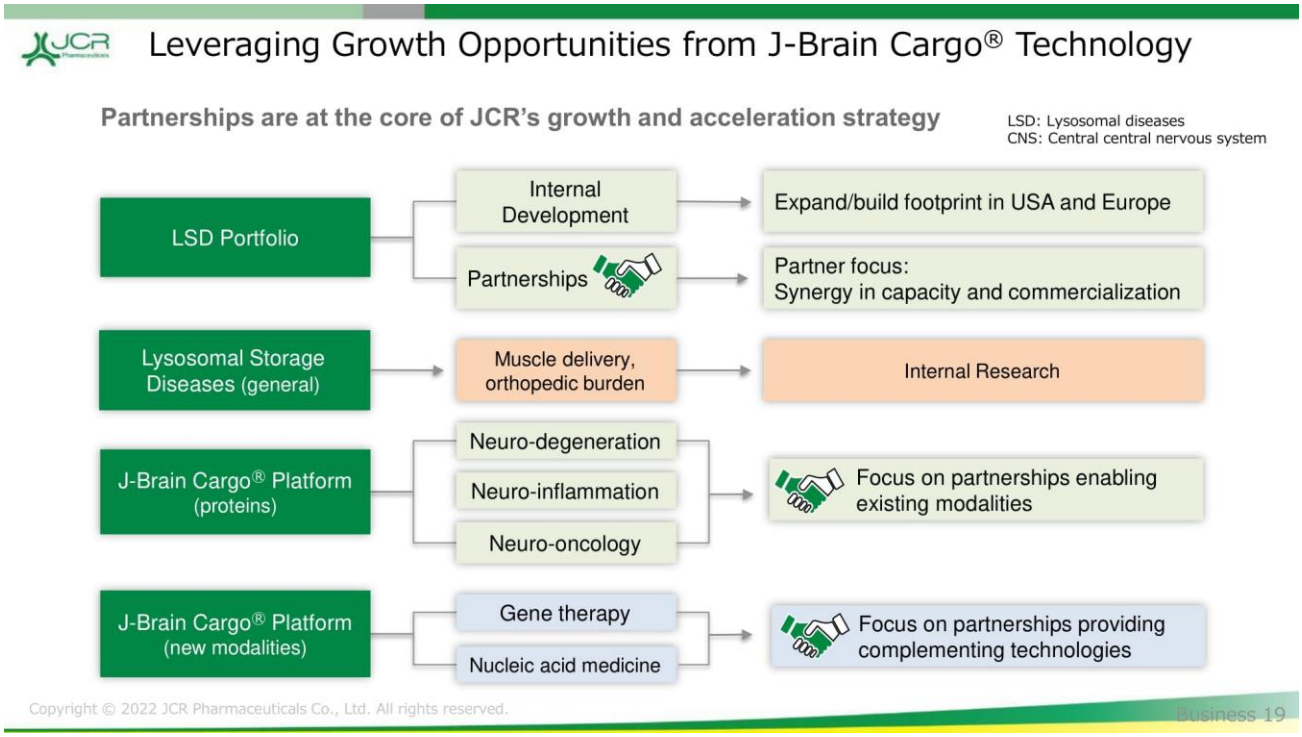
Growth Hormone area: Expected Timelines

★ Note: It is a plan at this stage and is subject to change
: progress in development stage since May 2022

Expected timeline (Growth Hormone area)

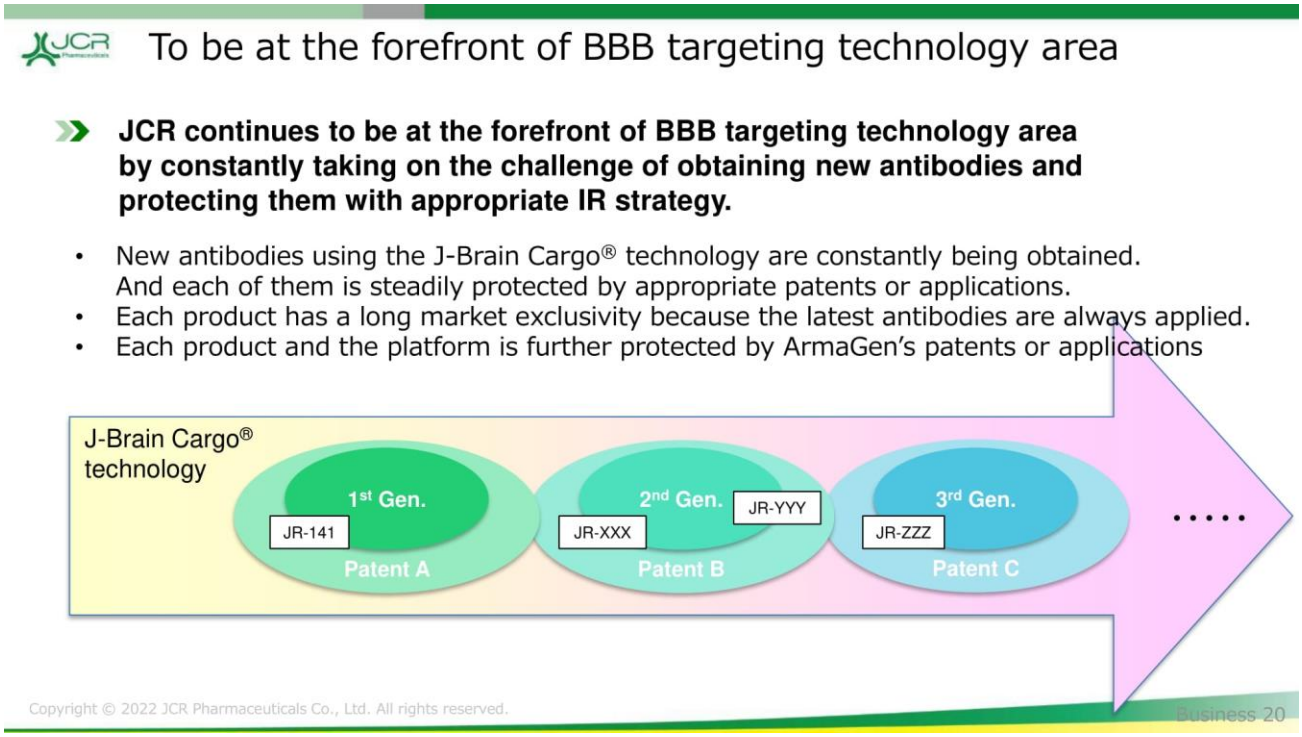
We have completed enrollment in our Phase II trial with a long-acting form of growth hormone JR-142. Considering the timelines of our growth hormone franchise, a global Phase III trial with JR-142 is expected to start in FY2023. We expect to have a decision on the acceptance of our marketing authorization application filing for JR-401X in summer next year.

Here, I would like to hand over to Sonoda-san.



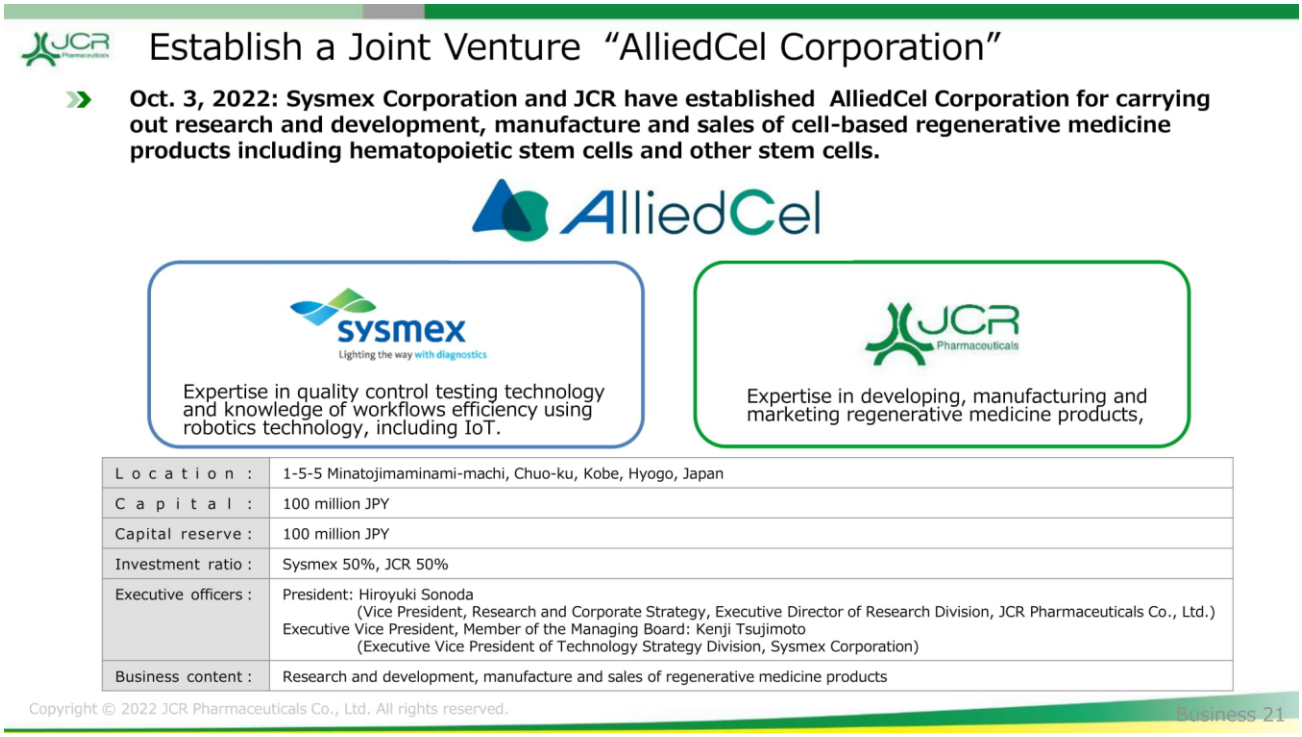
Sonoda: From here, I'd like to explain. So all of our LSDs, we have 18 compounds in the pipeline. Other than Fabry disease, we are using J-Brain Cargo technology, and this is quite unique. 18 compounds for us to internally develop is very difficult. Therefore, what we can do internally, that's what we are going to do. However, optimal is to also partner with other companies as well. Already for JR-141, we have collaboration with Takeda.

Other than LSD, so our platform technology J-Brain Cargo can be used. Therefore, how our technology can be used and expanded would be a critical point. Our pipeline or producing the products and also a placement in clinical studies would be a very difficult and tough area. Now, having the drug but the partners who do not have the technology to bring it to the brain, so we believe that our technology can optimize that. The good example is in the gene therapy area with Takeda, which could work for the CNS. We would like to make other examples like this.



To enable that, patent know-how is very important. J-Brain Cargo, there are so many antibodies, and they are constructing them. We are not using the initial antibody. We are gaining new antibodies because not only one antibody can be used for all the modalities. Being able to find the best antibodies is our know-how and also the experience that it would be based on, and that's our advantage, I believe.

As you can see in this figure, the first generation, second generation, and third generation, there are so many different types. In March, we have introduced this a little bit during the R&D explanation meeting. For each drug and modality, plus by fusing and also conjugating our J-Brain Cargo so as to fine-tune to the optimum state. J-Brain Cargo itself is a protein and how we can manipulate and optimize is very critical. We do have the base technology already.



This March, we made announcement about the joint venture established. This is together with Sysmex. We came up with the joint venture named AlliedCel. Sysmex as well as JCR, we have a research function in Kobe City that are located quite close to each other and have been discussing many things in the past. Strength of Sysmex and the strength of JCR, by combining them, we'll be able to come up with the product for the unmet needs area. With that in mind, we came up with this joint venture.

This is going to focus a certain area, which cannot be cured with the conventional product. By executing the strength of two companies, we would like to go for the research and development. At the end of the day, we'll be able to come up with the product to be launched at a quicker timing.

Moderator: Thank you very much for your kind attention. From now, we would like to have a Q&A session.

Question & Answer

Moderator [M]: The first question is from Citigroup Securities, Yamaguchi-san, please.

Yamaguchi [Q]: I'm Yamaguchi from Citi. The first question, in the presentation by CEO, the scheme of the contract. Ultra-rare disease, that's one thing and others, another. In some cases, they were separately, and some cases, they are together. For the future, ultra-rare and other diseases, you consider them as a block. And then, you have a contract with your companies for those blocks of your business. Am I right to say that?

Shin Ashida [A]: This is Ashida speaking. As we just mentioned, ultra-rare disease, unless you have a completely different scheme, it is not workable. Others, you cannot put them all together. We have about 11 diseases including them. In order to have a negotiation for each item, it is time consuming and very difficult. If it is possible, so the others, maybe we can compile them altogether, if there's any company who are able to handle that as a bulk, that's one way of a negotiation that we have just started. For the ultra-rare disease area, I talked about the investment to Mycenax and that was mentioned by Schmidt-san. For that as well, by using Mycenax and develop and manufacture the product, maybe that will be the scheme that we can go for, which is workable.

Yamaguchi [Q]: Thank you very much. I have another quick question. To develop ultra-rare disease, America FDA or EMA, I think that their cooperation is necessary. In order to access the process, I'm sure you have a certain scheme and by utilizing that scheme, do you think the earlier approval is possible?

Schmidt [A]*: Thank you for your question. If I understand it correctly, what you're saying is we need the cooperation from the regulatory authorities across different geographies in order to bring those ultra-rare disease assets to approval.

Yamaguchi [Q]*: Yeah.

Schmidt [A]*: We absolutely and cordially agree that we need to bring those agencies together, and we need to interact with the agencies probably even before the first scientific advice to educate them on the disease, on clinical endpoints, and on biomarkers that can be utilized for regulatory approval.

We also need to cooperate with the regulatory authorities to allow us to conduct innovative clinical trial designs, as it has been done for other ultra-rare diseases. One example I would like to mention here is MPS VII. Does that answer your question?

Yamaguchi [Q]: Yes. Thank you. I'd like to ask another question. There were 10 patients enrolled for IZCARGO and I thought the annual expectation was 20. I believe this is on-track progress. But with the unit price issue as well, I believe that the quarterly sales trend is quite flat or a little bit going down. But in the full year, do you expect to meet the budget?

Toru Ashida [A]: Let me take the question. As pointed out, for the full year, as we forecasted for H1 in terms of the dosing patients, it's progressing steadily and for full-year for 20 patients. But with this pace, so we believe that we can penetrate proper penetration with the patients. Thank you.

Moderator [M]: Yamaguchi-san, thank you. Continuing on, from Daiwa Securities, Hashiguchi-san, please.

Hashiguchi [Q]: This is Hashiguchi. Thank you. For IZCARGO, I have two questions. One is for the quarterly sales trend, as shown, the shipment from JCR is listed. But in terms of the sales, I'd like to know, there were 20 new patients. I believe that was the number. I'd like to know the number of discontinued patients.

And also in terms of sales, do I see it is actually growing in terms of sales?

Toru Ashida [A]: I will take this question as well. In terms of the actual consumption for Q2, it's actually 10 new patients. Full year, we are forecasting 20 patients. As for the actual consumption, we do not have the exact data on hand, but every month, the data that we get in terms of the actual consumption, that's also as expected in terms of the progress. Did I answer your question?

Hashiguchi [Q]: Yes. So once the treatment is started, what is the number of patients who stopped using it?

Toru Ashida [A]: In terms of the number, I'm afraid I cannot share the number here, but that is in the normal range of the dropout for normal pharmaceutical products.

Hashiguchi [Q]: Thank you. The second question, for future IZCARGO policy, for the attenuated patients, I believe that you will be looking into that as well. What's critical with attenuated patients? Is that the time for dosing? Both elaplace and IZCARGO are on a weekly infusion, I believe the infusion time would be adjusted depending on the situation. Those physicians and patients who are getting used to IZCARGO, what is the time of dosing?The dosage of IZCARGO is four times higher than that of elaplace. In terms of the time of dosing, is it four times? How many hours is it taking to dose the patients? If you have any actual practice information, can you share that as well?

Toru Ashida [A]: In terms of the other company's product, I cannot make the comment, but for IZCARGO, it's three hours to dose the patient.

Hashiguchi [Q]: And the time of dosing, the penetration into attenuated patients, what's your thought on the impact?

Toru Ashida [A]: Up to here in the actual clinical settings, for that, so there's been pointing out. But tenaciously, we are also working on promotion to the physicians.

Hashiguchi [M]: Thank you very much. That's it for me.

Moderator [M]: Thank you very much, Mr. Hashiguchi. The next question from Mitsubishi UFJ Morgan Stanley Securities, Ms. Kumagai, please.

Kumagai [Q]: Thank you. The first 141 global Phase III, the enrollment, would you please update that in light of the competitor, Denali?

Schmidt [A]*: This is Schmidt. Thank you for your question. The only quantitative public information that we have is a statement from Denali Therapeutics made at the MPS Family Conference in Barcelona on October 7. And at this conference, they announced that they dosed the first patient in the global Phase III study for DNL310 mid-September in the United States. If we compare this to the progress of the JR-141 global study, I think it is safe to say we are significantly ahead.

Kumagai [Q]: Second question about the contract. The President gave a little comment. But for the future, against the plan, the achievement of JPY15.4 billion, the number was given. Would you please elaborate more on that?

Shin Ashida [A]: The contract with regards to the license contract, we've been continuing to negotiate. First of all, our ultra-rare disease asset we'll be able to sign on the contract before long. But other LSDs, if it is one by one arrangement, we'll be able to do so quickly. However, we are now thinking about the scheme of the contract to integrate them all, putting them all together. That is going to be favorable for JCR for our future rather than going for one by one.

About the JR-142, we've started the negotiation as well. Next fiscal year, it will go into the Phase III. Therefore, we will be able to come up with a conclusion quicker than we expect.

Kumagai [Q]: I understood very clearly. Thank you very much.

Moderator [M]: Kumagai-san, thank you. From Morgan Stanley MUFG Securities, Muraoka-san, please.

Muraoka [Q]: Thank you. The first question I have is related to ultra-rare contract that you mentioned very near term, and that will be concluded and the interpretation of that is difficult, I think. Are you saying like within this year without waiting Q4? So is it likely to be concluded? Is that correct?

Shin Ashida [A]: Within this year, yes, surely, it will be concluded. You can interpret that way.

Muraoka [Q]: In that case, so JPY15.4 billion, do you think you'd get 1/2 of that?

Shin Ashida [A]: In terms of the content, I cannot share the details here. However, surely, it will be concluded by the end of this year.

Muraoka [Q]: Understood. Thank you very much. The other question, IZCARGO domestic sales, I just cannot really get it. You said as planned, so JPY5.2 billion annually for the plan. Right now, JPY2.1 billion and the rest of JPY3 billion in the next six months? I cannot really make it work. How can I actually understand this?

Toru Ashida [A]: Thank you very much. Right now, what I can share is not really precise information. We do have quite different age levels with the existing therapies and newly diagnosed patients. We are trying to get them on our IZCARGO as soon as possible, and that's our promotional activities.

For this year, new patients, we are targeting 20. Depending on that, the age as well as the weight of patients, the dose level changes, therefore, in terms of the sales, after the launch last year, and those who started dosing last year, they are continuing on, and they are on the basis as well.

Adding on, we have new patients. We do have quite a lot of pediatric patients. But as old as 50 years old, therefore, we have quite a broad range of weight difference and also dose difference. That's assumed in targeting 20 total new patients for this year. Therefore, from the sales perspective, we do believe we will achieve our target. Did I answer your question?

Muraoka [Q]: If you achieve 20 patients, you will be able to achieve JPY5.2 billion in the plan. Is that correct?**Toru Ashida [A]:** Yes. So with 20 new patients with IZCARGO, if the prescription starts, then yes, the sales target will be achieved, and that's our calculation.

Muraoka [Q]: You mentioned also lower age as well as the lower weight. And you don't have to down revise the target. Is that correct?

Toru Ashida [A]: Theoretically, where we cannot control, that might be a possibility. But regardless of age and weight, we'd like to dose IZCARGO to more patients, even one more patient and that's the activities that we are working on.

Muraoka [M]: Thank you. I understood.

Moderator [M]: Thank you, Muraoka-san. SBI Securities, Mr. Kawamura, please.

Kawamura [Q]: Thank you very much for your explanation. I'm Kawamura. About 171,, three or four case, I think it was increase in numbers. Thank you very much for your explanation. The secondary endpoint is mentioned, but the full data, when do you think you will be able to disclose the full data? I recognize that if

the HS in the CSF is reduced in a lysosomal disease development product using J-Brain Cargo, the clinical symptoms are also recovered.. Am I right? Clinically, it is going to be improved if the heparan sulfate is reduced. Would you please respond?

Schmidt [M]*:The patients are all now in Part Two of the clinical study. This is an open-label study, so we can look into the database at any time. We believe that we have a substantial set of clinical data available also on neurocognitive outcomes by next summer. However, our look into the current database status, I think it allows us an ability to already recognize significant patterns that allow us already to design the Phase III study and to identify clinically relevant endpoints.

What I also would like to emphasize here is the real vision and the potential of JR-171 is actually in the post-transplant population in MPS I. This constitutes like 65% of the entire MPS I population. And this population is cognitively struggling. We believe that JR-171 can bring a very significant improvement to those patients and thereby bring totally different dynamics into the MPS I market. I hope that answers your question.

Kawamura [Q]: Yes. Thank you very much. Finally, this is a question to Sonoda-san. LSD, I'd like to talk about the Fabry disease. A company come up with a good follow-up data, one-year data by gene therapy. Basically, you explained before that gene terapy and enzyme replacement therapy will cosxist. If there is any difference from what you stated before and now, are there any differences that you experience?

Sonoda [A]: Sonoda speaking. Gene therapy, I think what you're referring to data from with AgroBio or Sangamoor whatever, maybe you're talking about that. But actually, they came up with a good and positive result for patients, unlike what we expected. So this result itself, the enzyme concentration in blood is maintained at a certain level. But if we take a close look at it, it's not the case for all the patients.

So how long this maintenance of the level continue? That's a very important point. Another point is having to do with the safety. Those are the points. As of today, it is just too early for us to come up with a judgment. We need to see the long-term, the process and for increased number of patients.

For the second question to Schmidt-san, I will also answer. The question is whether the decrease of HS in CSF, and whether this can be directly linked to clinical outcomes. We would like to believe in that. I expect this will be the case since all developments use the same J-Brain Cargo technology.. Having said that, for each disease, there are some differences. Type of substrate accumulated in CSF, the volume, and then the material and what is accumulated in the brain, it may be different. And so in terms of inside the brain, only estimation, but for the MPS, there may be higher probability, as you mentioned.

Kawamura [M]: That's all from me. Thank you.

Moderator [M]: We apologize, even though I do see raising hands, we are approaching the end of the meeting, so we'd like to have them answered at individual meetings. Thank you.

For further requests, please reach out to Mr. Kitamura for PR and IR Office. Regarding this video, it will be posted in our website on a later date.

With this, for JCR Pharma term ending in March 2023, the Q2 financial results briefing is concluded. Thank you very much for your participation.

[END]

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