JCR Pharmaceuticals Co., Ltd.

Financial Results Briefing for the Fiscal Year Ended March 2021 Presentation

May 17, 2021

Event Summary

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[Number of Speakers]	3

Shin Ashida	Representative Director, Chairman, President, and CEO
Kazunori Tanizawa	Corporate Officer, Executive Director, Development Division
Yoshihiro Ohta	Director, Accounting Department, Management Division

Presentation

Ashida: I'm Shin Ashida from JCR Pharmaceuticals Co., Ltd. Thank you very much for watching this video of the financial results briefing for the fiscal year ending March 31, 2021. We would like to express our sincere gratitude to all of you for your continued support of our company.

Today's financial results briefing will be followed by an overview of the financial results for the fiscal year ending March 31, 2021 and an explanation of the R&D status from the respective directors.

In short, the financial results for the fiscal year ended March 31, 2021 recorded record highs, with both net sales and profits far exceeding the forecasts made at the beginning of the fiscal year.

Next, JR-141 was approved for production and marketing as IZCARGO in Japan in March and is scheduled to be listed on the National Health Insurance, or NHI, drug price list and begin sales on May 19. This is a very important milestone for us.

JR-141 is the first drug to use our proprietary technology, J-Brain Cargo. The results of long-term administration starting at from a Phase I/II 4-week study conducted in Japan and continuing for over 100 weeks to date have confirmed that there are no safety issues.

The results of these clinical trials have bolstered our confidence that J-Brain Cargo can be bring good results for those suffering from the approximately 16 lysosomal diseases for which treatments are currently under development. In addition, the clinical results showed very positive outcomes for both the patients and their families.

Next, I would like to talk about the license agreement for JR-141. We are continuing negotiations to conclude a contract with a company that is capable of selling our lysosomal therapeutics using J-Brain Cargo, such as JR-141, on a global scale.

Negotiations concern not only JR-141, but also with other compounds currently under development that use J-Brain Cargo technology. We are also considering the possibility of global marketing for diseases such as Sly syndrome and Sanfilippo syndrome, where the number of patients is small even when seen on a global scale.

Next, I would like to talk about bulk production of AstraZeneca vaccine solution.

As for the production of the vaccine solution, a considerable amount of manpower was put into the production from the beginning of this year, and the vaccine was successfully shipped in March. This vaccine solution has been licensed to companies in more than 10 countries around the world, and the quality of the solution we produce has been highly rated. With the ongoing coronavirus pandemic, we will continue to work hard, especially at manufacturing sites, to contribute to the stable supply of products in Japan as soon as possible.

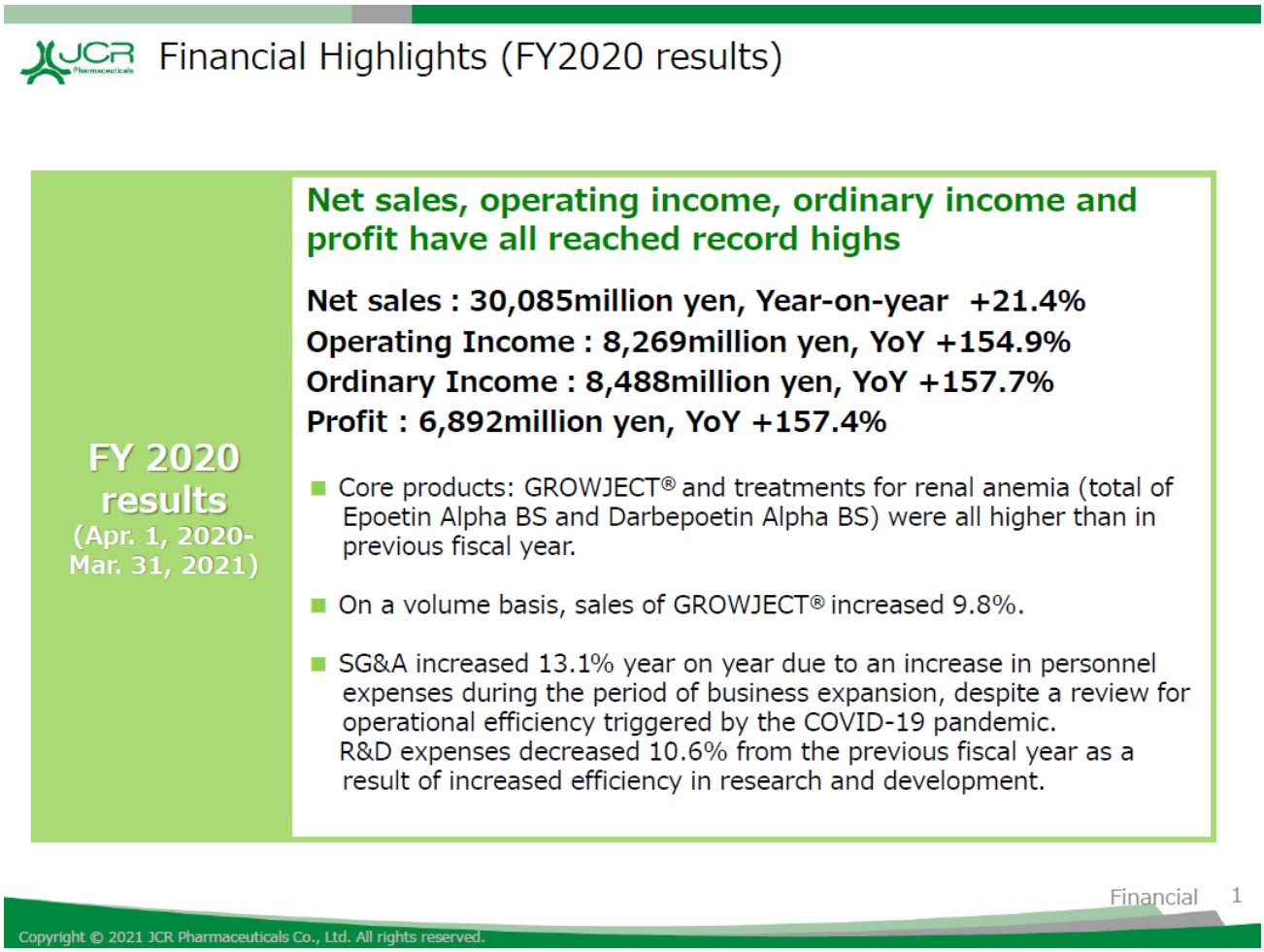
With regard to the 3-year plan, "Revolution", announced in May last year, sales and operating income for this fiscal year will be much higher than expected, but we will continue to work on important management issues.

Finally, I would like to talk about the new structure of the Board of Directors and organizational changes for the General Meeting of Shareholders to be held next month.

I feel that our young directors, including those who were newly appointed last year, are bringing new growth to the Company. As for the Company's organization, we will continue to implement organizational changes, a continuation of the changes made after the General Meeting of Shareholders last year. We are also working

on the development of the next fundamental technology to follow J-Brain Cargo. We believe this is essential for the Company's long-term growth.

I believe this is the next stage for JCR. The next generation of management and employees will lead the Company and continue to take on challenges as Team JCR. We would like to ask for your continued support.



Ohta: My name is Yoshihiro Ohta, and I am General Manager of the Accounting Department. I would like to present the results for the fiscal year ended March 31, 2021 and the forecast for the fiscal year ending March 31, 2022.

First, an outline of the financial results for the fiscal year ending March 31, 2021. Net sales, operating income, ordinary income, and current net income have all reached record highs.

Consolidated Results

(Unit: million yen)

Consolidated	FY2019 (Apr. 1, 2019-Mar. 31, 2020) A	FY 2020 (Apr. 1, 2020-Mar. 31, 2021) B	Year-on-Year (B-A)/A	Reference Initial forecast (before fixing)
Net sales	24,781	**30,085**	+21.4%	27,200
Cost of Sales	7,901	**7,812**	(1.1)%	6,800
Gross Profit	16,880	**22,272**	+31.9%	20,400
SG&A	7,638	**8,643**	+13.1%	8,000
R&D Expenses	5,997	**5,360**	(10.6)%	6,400
Operating Income	3,244	**8,269**	+154.9%	6,000
Ordinary Income	3,293	**8,488**	+157.7%	6,000
Profit*	2,678	**6,892**	+157.4%	4,800

*Profit attributable to owners of parent

Ratio of Cost of Sales	31.9%	**26.0%**	(5.9)%	25.0%
Ratio of Cost of R&D	24.2%	**17.8%**	(6.4)%	23.5%
Operating Profit Ratio	13.1%	**27.5%**	+14.4%	22.1%

(Reference)

R&D expenses**	6,582	**5,856**	(11.0)%	7,600

**R&D expenses before deducting contribution amount by collaborative R&D destinations

Financial 2

Net sales totaled JPY30.085 billion, an increase of 21.4% from the previous fiscal year. The details will be explained later.

Total selling, general, and administrative expenses increased by 2.7% from the previous fiscal year. Selling, general, and administrative expenses increased 13.1% year on year to JPY8.643 billion due to an increase in personnel expenses during the period of business expansion. This despite a review of operational efficiency triggered by the coronavirus pandemic.

Research and development expenses decreased by 10.6% from the previous fiscal year to JPY5.36 billion as a result of increased efficiency in research and development. The schedule is progressing as planned. The ratio of R&D expenses to net sales was 17.8%.

As a result, operating income increased 154.9% from the previous fiscal year to JPY8.269 billion, ordinary income increased 157.7% to JPY8.488 billion, and current net income increased 157.4% to JPY6.892 billion.

The cost of sales ratio was 26%, an improvement of 5.9 percentage points from the previous fiscal year. The ratio of operating income to net sales was 27.5%, an improvement of 14.4 percentage points from the previous fiscal year.

Breakdown of net sales (Consolidated)

(Unit: million yen)

	FY2019 (Apr. 1, 2019- Mar. 31, 2020) A	Composition ratio	**FY2020 (Apr. 1, 2020- Mar. 31, 2021) B**	**Composition ratio**	**Year-on-Year (B-A)/A**
GROWJECT®	12,650	51.0%	**13,256**	44.1%	+4.8%
Treatments for renal anemia	5,509	22.2%	**7,087**	23.6%	+28.6%
Epoetin Alpha BS Inj. [JCR]	4,097	16.5%	**3,278**	10.9%	(20.0)%
Darbepoetin Alpha BS Inj. [JCR]	1,412	5.7%	**3,809**	12.7%	+169.7%
TEMCELL® HS Inj.	3,126	12.6%	**2,441**	8.1%	(21.9)%
Agalsidase Beta BS I.V. Infusion [JCR]	317	1.3%	**470**	1.6%	+48.2%
Total Core products	**21,602**	**87.2%**	**23,254**	**77.3%**	**+7.6%**
AZD1222 bulk	–	–	**404**	1.3%	—
License Revenue	2,050	8.3%	**6,406**	21.3%	+212.4%
Other	1,125	4.5%	**18**	0.1%	(98.3)%
Total Net Sales	**24,781**	**100.0%**	**30,085**	**100.0%**	**+21.4%**

Financial 3

I will now explain the breakdown of net sales.

First, sales of our mainstay product, Growject, a recombinant human growth hormone, increased 4.8% year on year to JPY13.256 billion. On a volume basis, sales increased by 9.8%, showing a steady increase.

Sales of renal anemia treatments, our other mainstay product, also increased 28.6% year on year to JPY7.087 billion. Sales of Temcell, a regenerative medicine product, decreased by 21.9% from the previous fiscal year due to partial shipment restrictions. These were implemented in April 2020 to build up inventory. Sales of Agalsidase beta, a treatment for Fabry disease, increased by 48.2% from the previous fiscal year.

In addition, sales of AZD1222 bulk vaccine solution for the coronavirus vaccine, which we were entrusted with from AstraZeneca, amounted to JPY404 million. Contract revenue totaled JPY6.406 billion, an increase of JPY4.356 billion from the previous fiscal year.

As a result of the above, total net sales increased by 21.4% YoY to JPY30.085 billion, achieving a record high.

Balance Sheet (Consolidated)

(Unit: million yen)

	Mar. 2020	**Mar. 2021**	Main Increase/decrease		Mar. 2020	**Mar. 2021**	Main Increase/decrease
Current assets	28,342	**48,545**	Cash and deposits +15,287 Accounts receivable-trade +206 Inventories +4,699	**Current liabilities**	10,434	**29,028**	Short-term loans payable +7,970 Special suspense account for tax purpose reduction entry +3,828
				Non-current liabilities	4,761	**6,199**	Long-term loans payable +950
Non-current assets	19,433	**25,238**	Property, plant and equipment +2,297 Patent right +2,988	**Total liabilities**	15,195	**35,227**	+20,031
				Total net assets	32,579	**38,557**	Profit etc. +5,977
Total	47,775	**73,784**	+26,008	**Total**	47,775	**73,784**	+26,008
Capital investment	5,296	**3,965**		**Equity ratio**	66.6%	**51.3%**	

Financial 4

The following is an explanation of our financial position as of the end of fiscal year 2021.

Total assets amounted to JPY73.784 billion, an increase of JPY26.008 billion from the previous fiscal year. Current assets increased by JPY20.203 billion over the previous fiscal year, mainly due to an increase in cash and deposits and inventories.

The main reason for the increase in inventories in the midst of the coronavirus pandemic is the procurement of materials early on to ensure stable production.

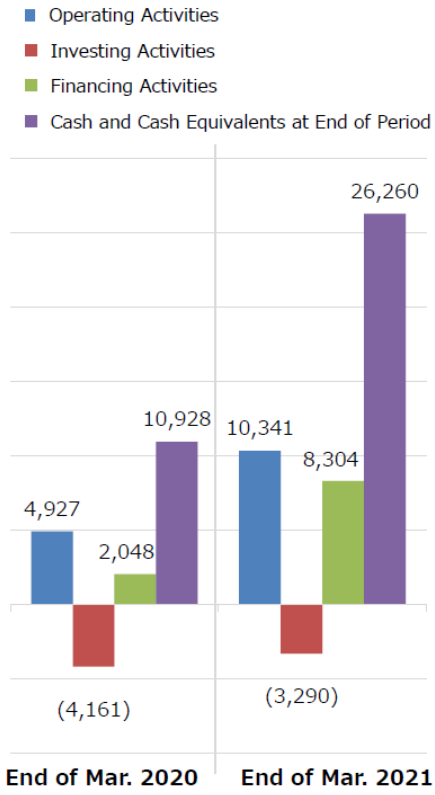
Fixed assets increased by JPY5.805 billion over the previous fiscal year. This was mainly due to the acquisition of land for the construction of a new plant for production of coronavirus vaccine solution, as well as the acquisition of ArmaGen, Inc.

Total liabilities increased by JPY20.031 billion from the previous fiscal year to JPY35.227 billion, mainly due to an increase in loans payable. The aim is to supplement the balance of cash equivalents in light of the spread of the coronavirus pandemic.

Net assets increased by JPY5.977 billion from the previous fiscal year to JPY38.557 billion, mainly due to the recording of net income.

Cash Flows (Consolidated)

(Unit: million yen)



	End of Mar. 2020 A	**End of Mar. 2021 B**	Year-on-year B - A
Income before income taxes	3,422	**8,653**	5,231
Depreciation and amortization	1,434	**1,892**	458
Accounts payable-trade	92	**2,253**	2,161
Inventories	258	**(4,699)**	(4,957)
Other	(280)	**2,241**	2,521
Operating Activities	4,927	**10,341**	5,413
Securities	668	**147**	(521)
Capital investment	(4,838)	**(4,780)**	58
Other	8	**1,342**	1,334
Investing Activities	(4,161)	**(3,290)**	870
Loans payable	3,200	**9,420**	6,220
Cash dividends paid/ treasury stock	(973)	**(1,069)**	(96)
Other	(177)	**(46)**	131
Financing Activities	2,048	**8,304**	6,255
Cash and Cash Equivalents at End of Period	10,928	**26,260**	15,332

Financial 5

Next, I will explain the status of the statement of cash flows.

Cash flow provided by operating activities increased by JPY5.413 billion from the previous fiscal year to JPY10.341 billion. Cash flow used in investing activities decreased by JPY870 million from the previous fiscal year to JPY3.29 billion. Net cash provided by financing activities increased by JPY6.255 billion from the previous fiscal year to JPY8.304 billion. As a result, the balance of cash equivalents at the end of the fiscal year increased by JPY15.332 billion from the previous fiscal year to JPY26.26 billion.

The above is an explanation of our business results for the fiscal year ending March 31, 2021.

Forecast Highlights (FY2021)

Earnings Forecast
(Apr. 1, 2021-Mar. 31, 2022)

Net sales : 49,000million yen, Year-on-year + 62.9%
Operating income : 18,700million yen, YoY +126.1%
Ordinary income : 18,700million yen, YoY +120.3%
Profit : 13,300million yen, YoY +92.9%

- Topics of core products: The launch of the IZCARGO® and growth in sales volume of GROWJECT®
  - Develop more effective and efficient information provision activities for each product by changing the sales structure.
  - Differentiation in Growth Hormone field by improving treatment satisfaction, taking advantage of the characteristics of electric injection.
  - IZCARGO®
    Japan : Expected to be listed on the National Health Insurance (NHI) drug price list in May, 2021.
- Net sales is forecast to increase 62.9%, including active efforts in the licensing business and production of AZD 1222 bulk.
- Accordingly, operating income is forecast to increase 126.1% after absorbing increase in SG&A and R&D expenses.
  - SG&A is forecast to increase 35.4%
  - R&D is forecast to increase 92.2%

I would like to continue with the earnings forecast for the fiscal year ending March 31, 2022.

Topics for the current fiscal year include the launch of the IZCARGO, Hunter's syndrome drug, as well as aggressive efforts in the licensing business. Additionally, production of AZD1222 bulk solution is expected to make a significant contribution to earnings.

FY2021 Forecast (Consolidated)

(Unit: million yen)

Consolidated	FY2020 (Apr. 1, 2020- Mar. 31, 2021) A	FY2021 forecast (Apr. 1, 2021- Mar. 31, 2022) B	Increase/ decrease B - A	Year-on-Year (B-A)/A
Net Sales	30,085	**49,000**	+18,915	+62.9%
Cost of sales	7,812	**8,300**	+488	+6.2%
Gross profit	22,272	**40,700**	+18,428	+82.7%
SG&A	8,643	**11,700**	+3,057	+35.4%
R&D	5,360	**10,300**	+4,940	+92.2%
Operating Income	8,269	**18,700**	+10,431	+126.1%
Ordinary Income	8,488	**18,700**	+10,212	+120.3%
Profit*	6,892	**13,300**	+6,408	+92.9%

*Profit attributable to owners of parent

Ratio of Cost of Sales	26.0%	**16.9%**	(9.10)%	
Ratio of Cost of R&D	17.8%	**21.0%**	+3.20%	
Operating Profit Ratio	27.5%	**38.2%**	+10.70%	

(Reference)

R&D expenses**	5,856	**11,080**	+5,223	+89.2%

**R&D expenses before deducting contribution amount by collaborative R&D destinations

Financial 7

On the other hand, R&D expenses are expected to increase significantly by 92.2% YoY to JPY10.3 billion, mainly due to progress in R&D related to lysosomal disease therapeutics. Selling, general, and administrative expenses are also expected to increase in the midst of business expansion and are planned to increase by 35.4% YoY to JPY11.7 billion.

Accordingly, we plan to achieve significant increases in both sales and profits, with net sales of JPY49 billion, up 62.9% from the previous fiscal year; operating income of JPY18.7 billion, up 126.1%; ordinary income of JPY18.7 billion, up 120.3%; and net income of JPY13.3 billion, up 92.9%.

FY2021 Forecast (Consolidated)

(Unit: million yen)

Name of Business segment	FY2020 (Apr. 1, 2020-Mar. 31, 2021) A	Composition ratio	FY2021 forecast (Apr. 1, 2021-Mar. 31, 2022) B	Composition ratio	Increase/ decrease (B-A)
GROWJECT®	13,256	44.1%	13,900	28.4%	+644
Treatments for renal anemia	7,087	23.6%	6,400	13.1%	(687)
Epoetin Alpha BS Inj. [JCR]	3,278	10.9%	2,700	5.5%	(578)
Darbepoetin Alhpa BS Inj. [JCR]	3,809	12.7%	3,700	7.6%	(109)
TEMCELL® HS Inj.	2,441	8.1%	3,200	6.5%	+759
Agalsidase Beta BS I.V. Infusion [JCR]	470	1.6%	800	1.6%	+330
IZCARGO®	0	0.0%	2,800	5.7%	+2,800
Total Core Products	**23,254**	**77.3%**	**27,100**	**55.3%**	**+3,846**
AZD1222 bulk	404	1.3%	14,700	30.0%	+14,296
License Revenue	6,406	21.3%	7,200	14.7%	+794
Other	18	0.1%	0	0.0%	(18)
Total Net Sales	**30,085**	**100.0%**	**49,000**	**100.0%**	**+18,915**

Financial 8

Next, I would like to explain our forecast for net sales by product category for the fiscal year ending March 31, 2022.

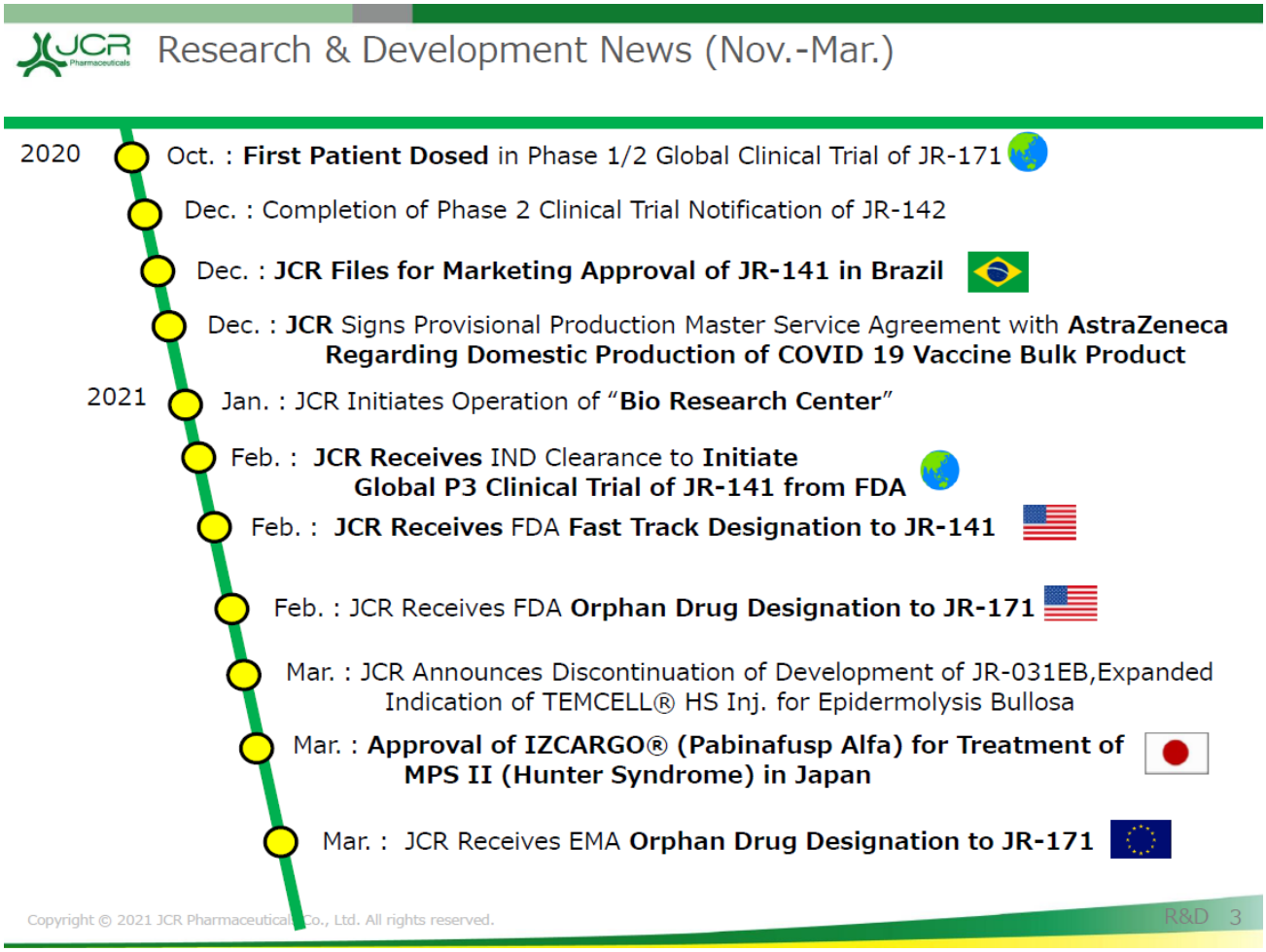
With respect to our mainstay product, Growject, we will strive to increase sales volume by developing more efficient and effective information provision activities for each product through changes in our sales structure. We will also aim to differentiate our products by improving treatment satisfaction, taking advantage of the characteristics of full-automatic electronic devices.

As a result, we forecast an increase of JPY644 million, up to JPY13.9 billion.

On the other hand, the forecast for renal anemia drugs is JPY6.4 billion, a decrease of JPY687 million from the previous fiscal year. We also forecast an increase in sales of Temcell by JPY759 million YoY, up to JPY3.2 billion. Similarly, we forecast sales of Agalsidase beta will increase by JPY330 million YoY to JPY800 million. IZCARGO is expected to be listed on the NHI drug price list this month, May 2021, and we forecast sales of JPY2.8 billion.

In addition, we forecast sales of JPY14.7 billion for AZD1222 solution. We also forecast an increase in contract revenue of JPY794 million over the current fiscal year, bringing the figure to JPY7.2 billion.

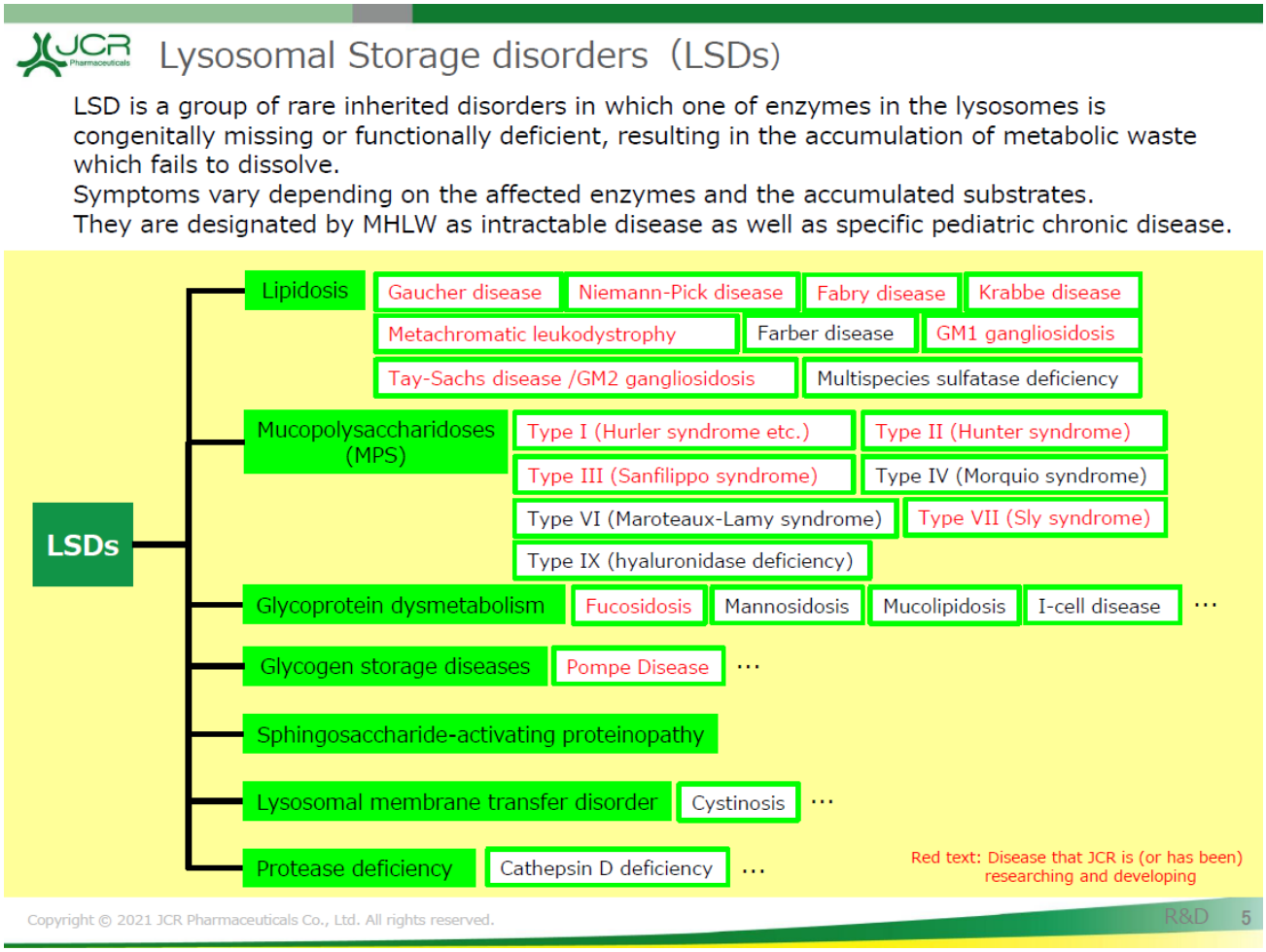
This concludes my presentation of the results for the fiscal year ended March 31, 2021 and the forecast for the fiscal year ending March 31, 2022. Thank you very much.



Tanizawa: I'm Kazunori Tanizawa, from the Development Division. I would like to explain the progress of our research and development.

First, I would like to share with you the highlights of our press release.

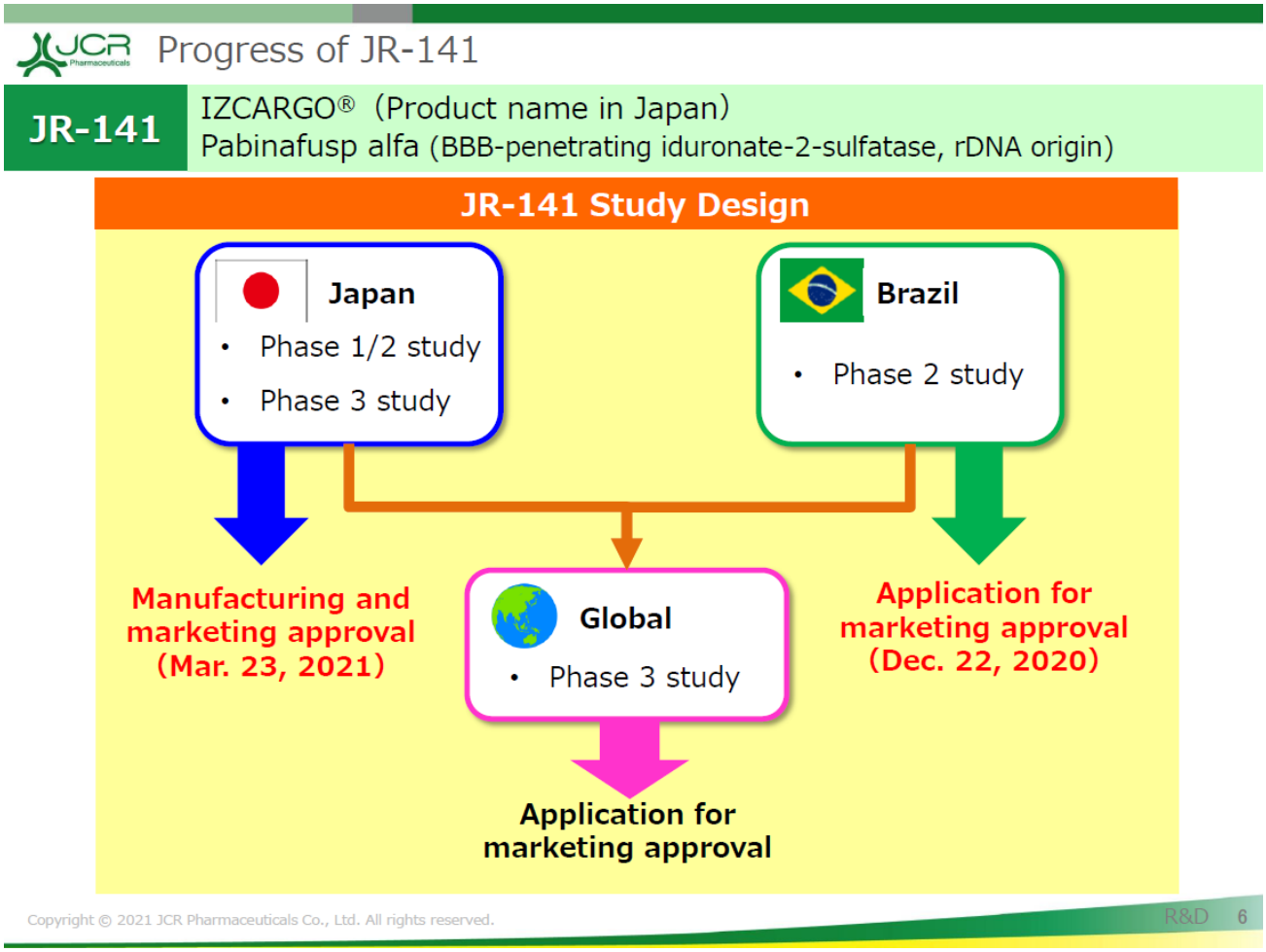
One of the major points is that IZCARGO obtained manufacturing and marketing approval in March. JR-171, an enzyme for the treatment of Hurler's syndrome, is also progressing well in Phase I trials.



First, I would like to explain the development status of JR-141.

Lysosomal diseases are a type of inborn error of metabolism, and are classified as lipidosis, mucopolysaccharidosis, or glycogenosis, depending on the substrate that accumulates.

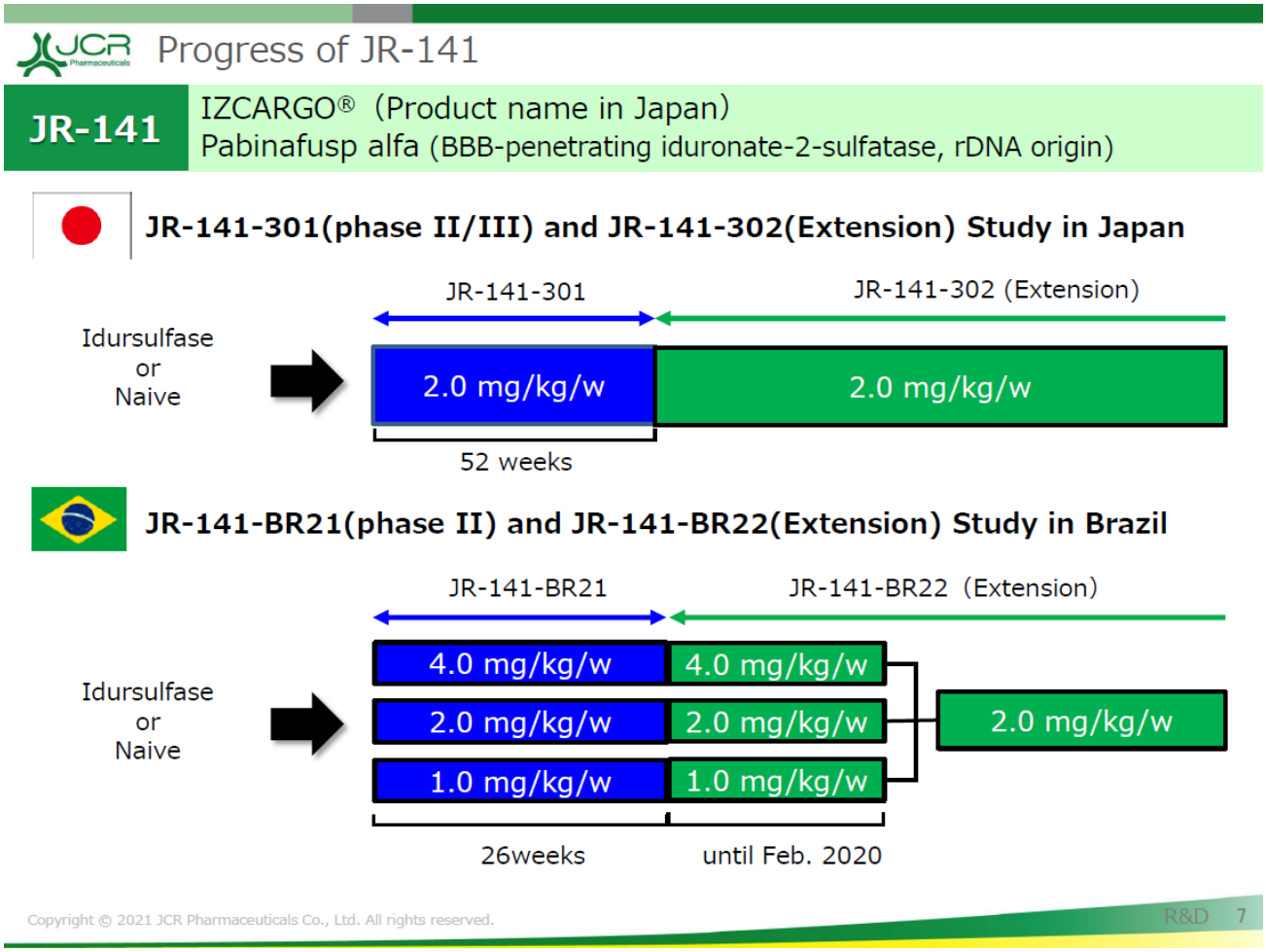
JR-141 targets Hunter syndrome, a type II mucopolysaccharidosis, while JR-171 targets Hurler syndrome, a type I mucopolysaccharidosis.



The overall view of development of JR-141 is shown here.

We have conducted Phase I/II clinical trials as well as and Phase III trials in Japan and have recently obtained approval. In Brazil, we have conducted Phase II trials and are currently in the process of reviewing an application for manufacturing and marketing approval by Brazilian healthcare regulatory authority.

We will be conducting global Phase III trials in the future, and we have already received a letter from the FDA in February this year, indicating that the IND has been accepted. As a result, we are currently preparing for the start of these trials.



Here is a general overview of clinical trials.

Phase III trials in Japan have progressed without issue, and we are still administering the drug at the approved dose of 2 mg/kg.

In Brazil, 3 doses of 1 mg, 2 mg, and 4 mg were studied, and in the end, from the perspective of efficacy and safety, 2 mg was selected as the optimal dose. Administration is continuing here as well.

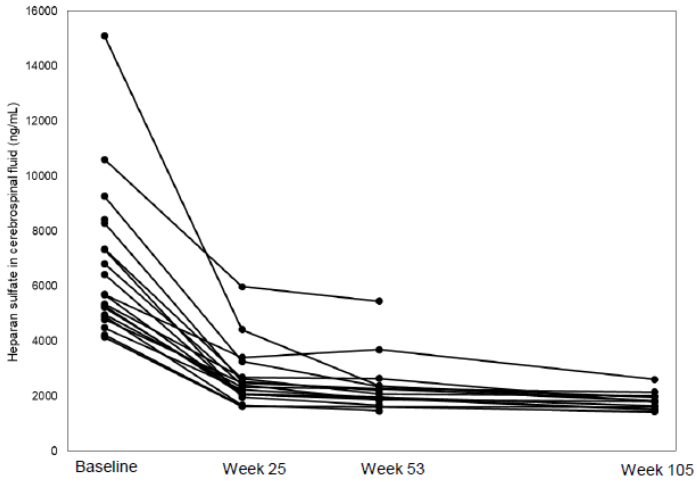
Progress of JR-141

JR-141

IZCARGO® (Product name in Japan)
Pabinafusp alfa (BBB-penetrating iduronate-2-sulfatase, rDNA origin)

Phase 3 trial･Extension study(JR-141-301/302) : Results

- **Time course of Heparan Sulfate (HS) in CSF (severe)**



- The concentration of substrates in CSF decreased in all subjects after 53 or 105 weeks of JR-141 administration, confirming that JR-141 passes through the blood-brain barrier(BBB) and has a long-term substrate degrading effect in the central nervous system

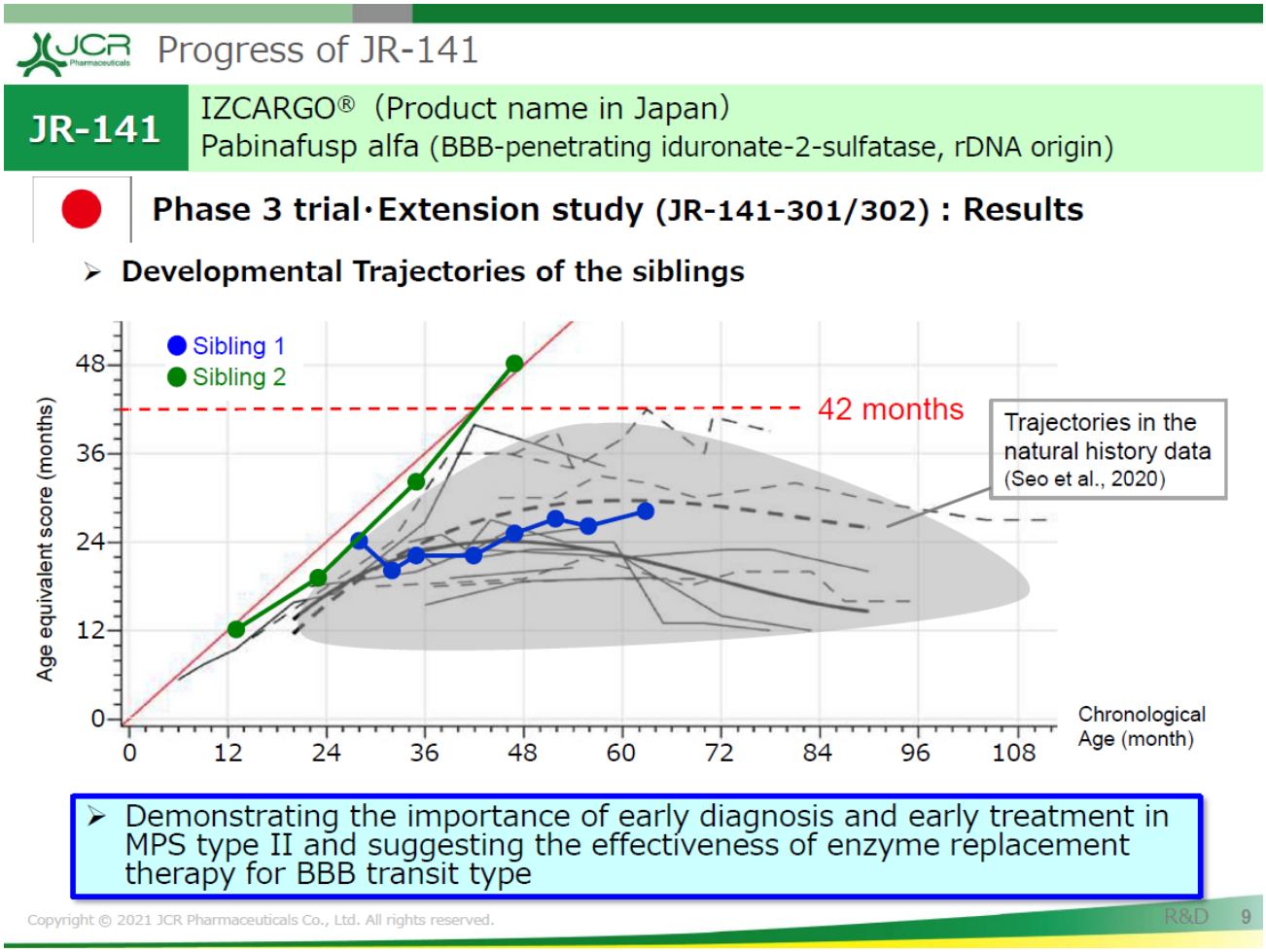
In the next slide, I will show you the data.

First, shown here is the trend of heparan sulfate concentration in the cerebrospinal fluid over time. This data came from severe forms.

As you can see, there was a decrease in heparan sulfate in all patients over 52 weeks.

We also have data from patients who have received the drug for 2 years, and we found that the concentration of heparin sulfate decreased further during 52 weeks in some cases. Where the concentration had fallen sufficiently and maintained at the 52-week concentration. As you can see, the trend continues for the 2 years.

We believe that it is very important to maintain the heparan sulfate concentration at the reduced level, which is equivalent to that seen in a mild form of the disease. As you can see from this data, JR-141 can suppress heparan sulfate in the long term.



This slide shows the importance of keeping heparan sulfate at a low level.

This green line shows the developmental progress of a patient who started JR-141 treatment at approximately 1 year and 11 months of age.

As you can see, the patient's developmental age is 48 months, so he is continuing in normal development. The concentration of heparan sulfate in CSF in this patient was less than 2,000 ng/mL. This suggests the importance in treatment of maintaining a very low level of heparan sulfate.

This blue line is the data of this patient's brother.

This patient had been undergoing existing enzyme therapy. In the background, the natural history of severe type of patients is shown in gray. Since this is a natural history, we can say that the development of the child slowed down from about 2 years old, which is different from that of his younger brother who received JR-141 treatment.

From this point of view, I would like to say that early diagnosis and early treatment are very important for disease management.

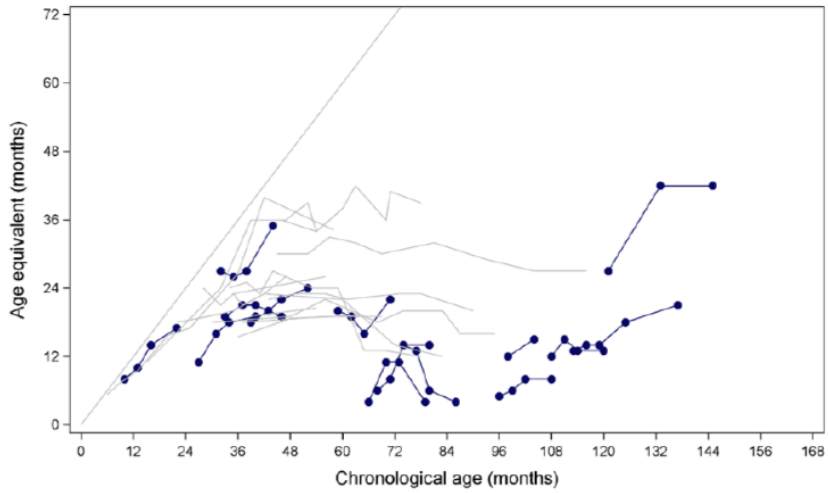
Progress of JR-141

JR-141

IZCARGO® (Product name in Japan)
Pabinafusp alfa (BBB-penetrating iduronate-2-sulfatase, rDNA origin)

Phase 2 trial (JR-141-BR21/BR22) : Results

- **developmental assessment (BSID-III)**



- Developmental assessment was stabilized or improved, suggesting that JR-141 may improve central nervous system symptoms in MPS type II

Next, this is the long-term developmental trend seen in the Brazilian study.

In this trial, we used the Bayley-III developmental questionnaire to assess developmental age. The Bayley-III is the standard questionnaire for assessing development in mucopolysaccharidoses and lysosomal diseases.

As you can see, it is an early age. Whether the age group is under 5 years old, over 5 years old, or over 10 years old, there is a clear indication of improvement or maintenance in developmental age.

Also, I would like to emphasize that we are considering recruiting patients who are less than 72 months old for the next global Phase III trial. The questionnaire used is the Bayley III, which is also included in the primary endpoint. Therefore, I would like to emphasize that the results of this study are also important because they can be used to predict the results of future Phase III trials.

JR-141 Global development

JR-141

IZCARGO® (Product name in Japan)
Pabinafusp alfa (BBB-penetrating iduronate-2-sulfatase, rDNA origin)

Global Phase 3 trial (JR-141-GS31) : Brief Summary

Countries : USA, Brazil, EU (Germany, France, UK)

Objective : To show efficacy on CNS and systemic symptoms.

Design :

- **2 cohorts, standard of care controlled, parallel-group trail**
- Target number of patients : 50 (Male)

	Subjects	Standard of Care	JR-141	Duration
CohortA	• **Neuronopathic patients** • 36-71 months old, IQ=55-75 • 30-35 months old, mutation in the IDS gene, judged the severe phenotype	**15**	**15**	105 weeks
CohortB	• **Attenuated patients** • >6 years old, IQ≧70	**10**	**10**	53 weeks

Endpoints :

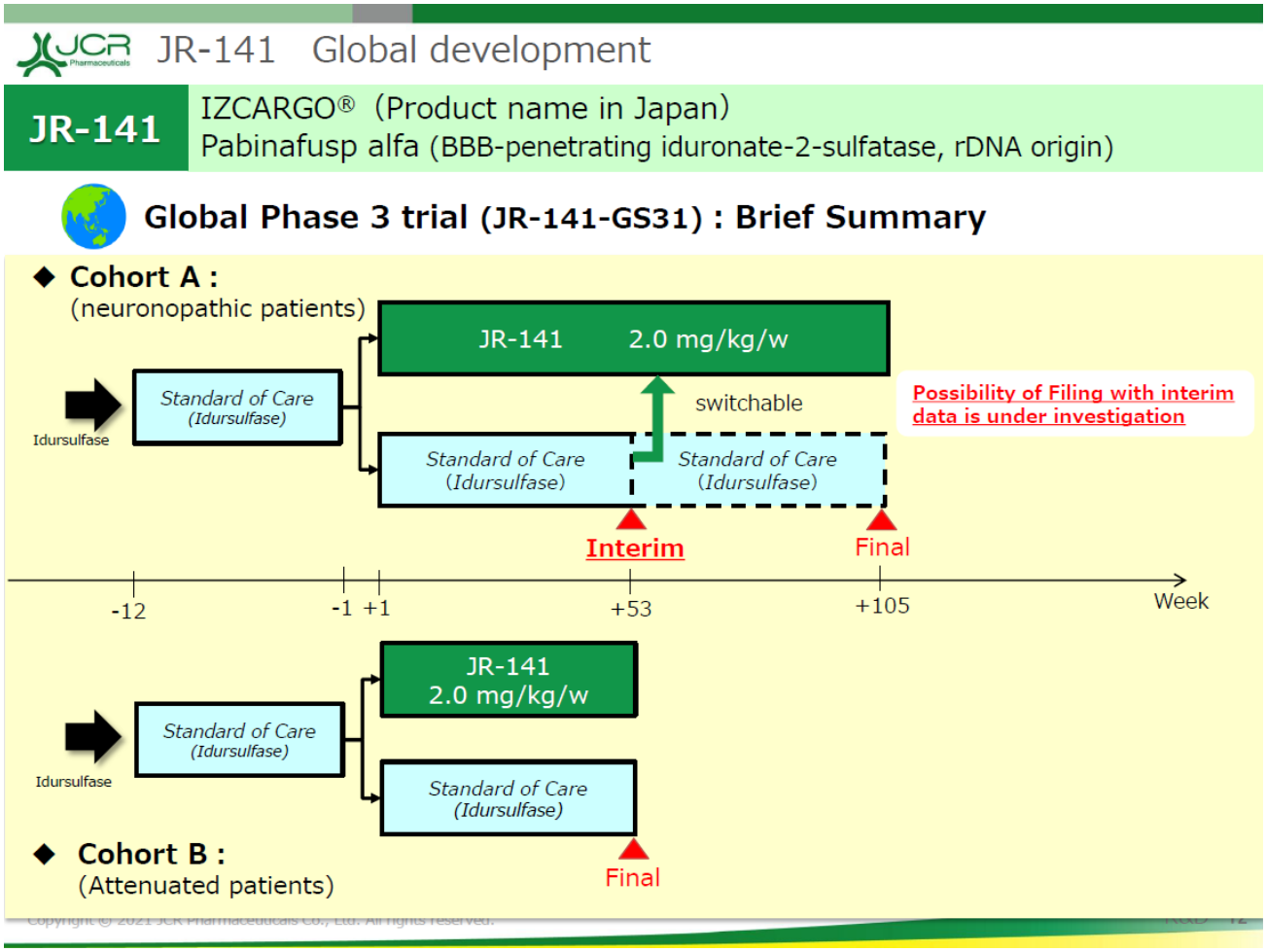
- HS in CSF, CNS symptoms (cognitive)
- Physical symptoms (liver volume, 6-minute walk)

ClinicalTrials.gov : Identifier : NCT04573023

Now, I would like to give you an overview of the global Phase III trial.

The countries scheduled for implementation are the United States, Brazil, Germany, France, and the United Kingdom. One of the main features of this exam is that it has 2 cohorts. One is Cohort A for Neuronopathic patients, and the other is Cohort B for Attenuated patients. We are planning to recruit a total of 50 patients.

As for the primary endpoints, we have not set a single primary endpoint, but plan to evaluate multiple items, such as the concentration of heparan sulfate in the cerebrospinal fluid, evaluation of central nervous system symptoms, and evaluation of systemic symptoms.



The trial design is shown here.

As I mentioned earlier, in Cohort A, the trial will continue for 2 years in Neuronopathic patients. Cohort B is a 1-year study of patients with mild forms of the disease.

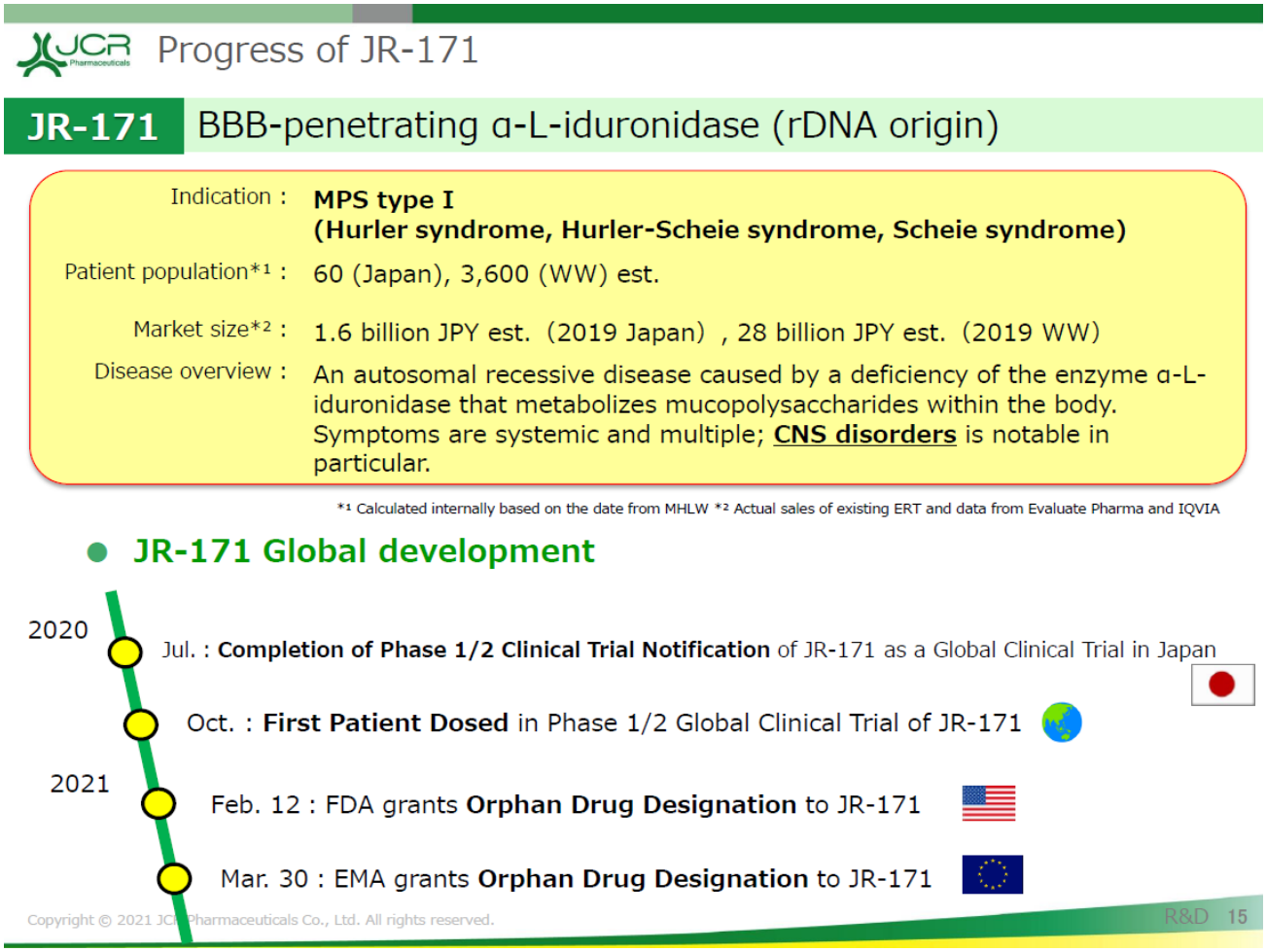
In each case, the interim analysis will be conducted within one year, so the primary endpoint that I explained earlier will be examined in comparison with the target group at this interim analysis stage.

Depending on the results of that, we may consider applying marketing approval submission based on the interim analysis.

This shows the global development status.

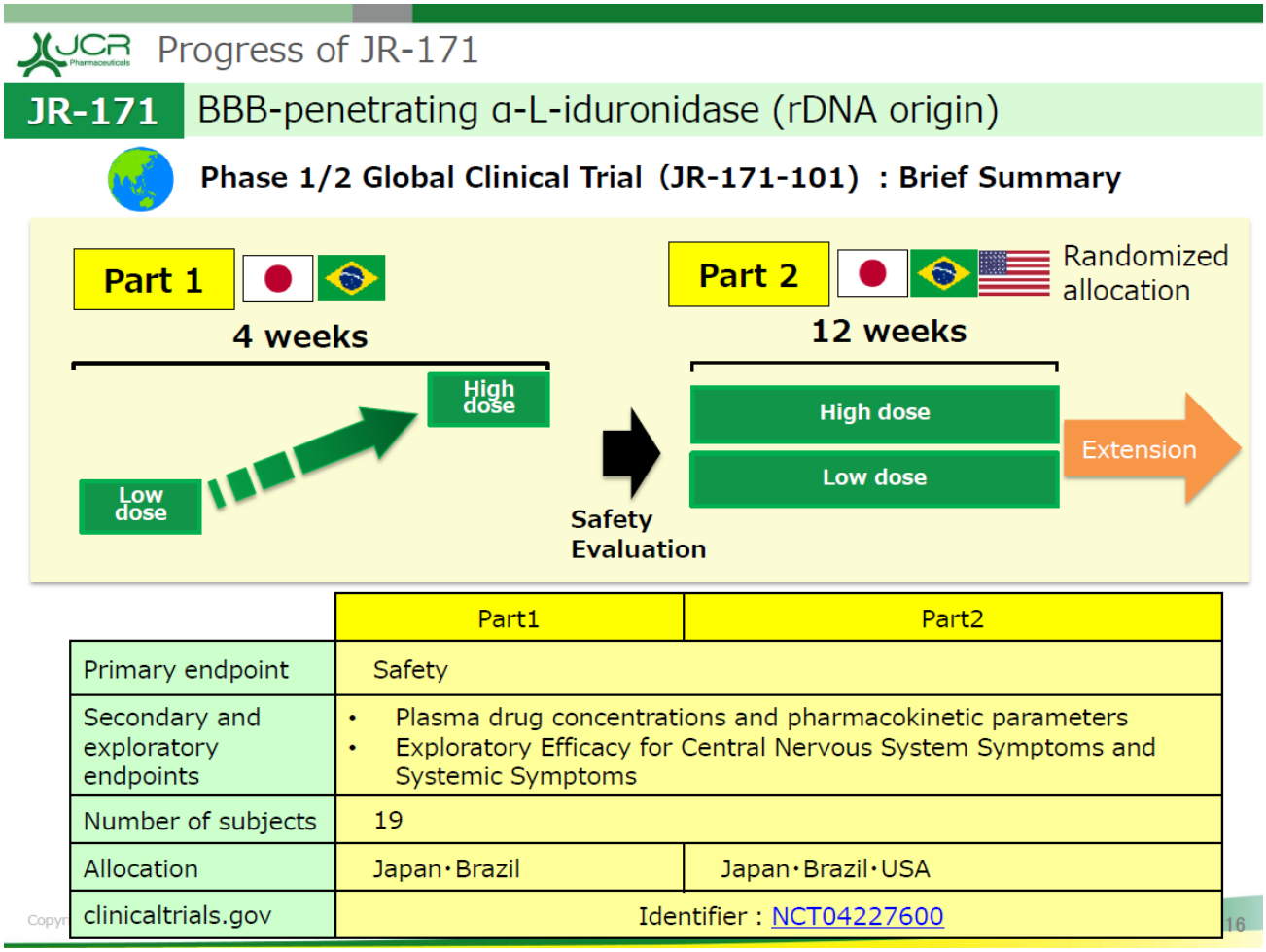
As I mentioned earlier, we obtained manufacturing and marketing approval in Japan in March. In addition, the drug is scheduled to be listed on the NHI drug price list in May.

As for Brazil, we submitted an application in December last year and are still in discussions with the ANVISA. If all goes according to plan, we should be able to start global Phase III trials in 2021. The trial should be able to start soon.



Next, I would like to explain the development status of JR-171.

JR-171 is a treatment for the mucopolysaccharidosis type I disease, Hurler syndrome. This has already been designated as an orphan drug this fiscal year, and our Phase I/II clinical trials are progressing smoothly, so I would like to explain the details.



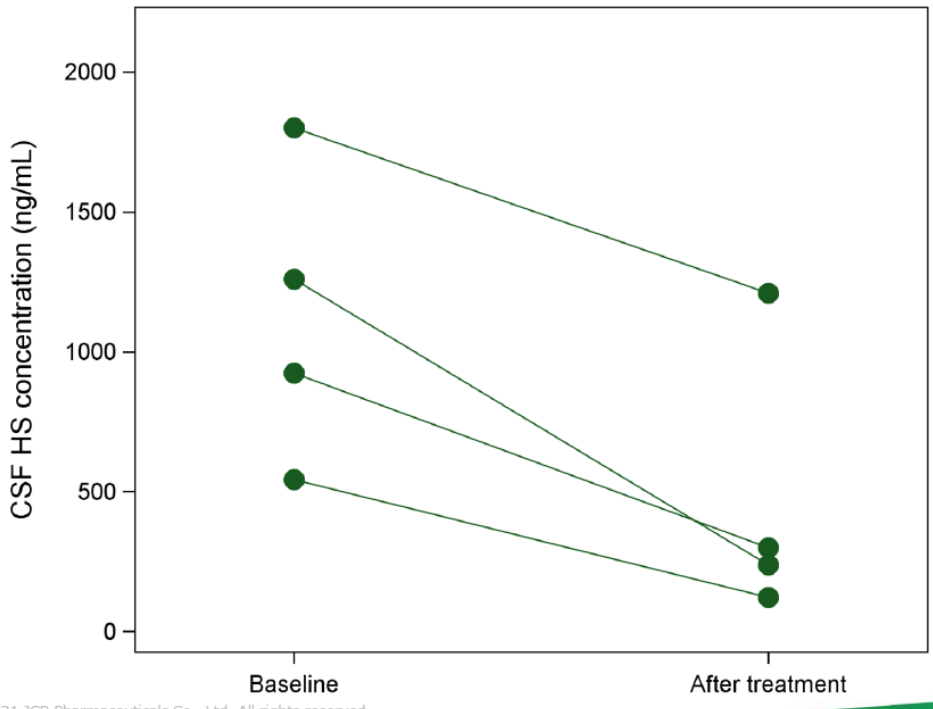
Here is the study outline for the global Phase I/II study.

In Part 1, we will study the safety of dosage and administration, and in Part 2, we will study the long-term effects of low dose and high dose. The PK/PD profile and other factors will be confirmed in this study. Part 1 has already been completed, and we have obtained data on heparan sulfate, which I would like to show in the next slide.

Progress of JR-171

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

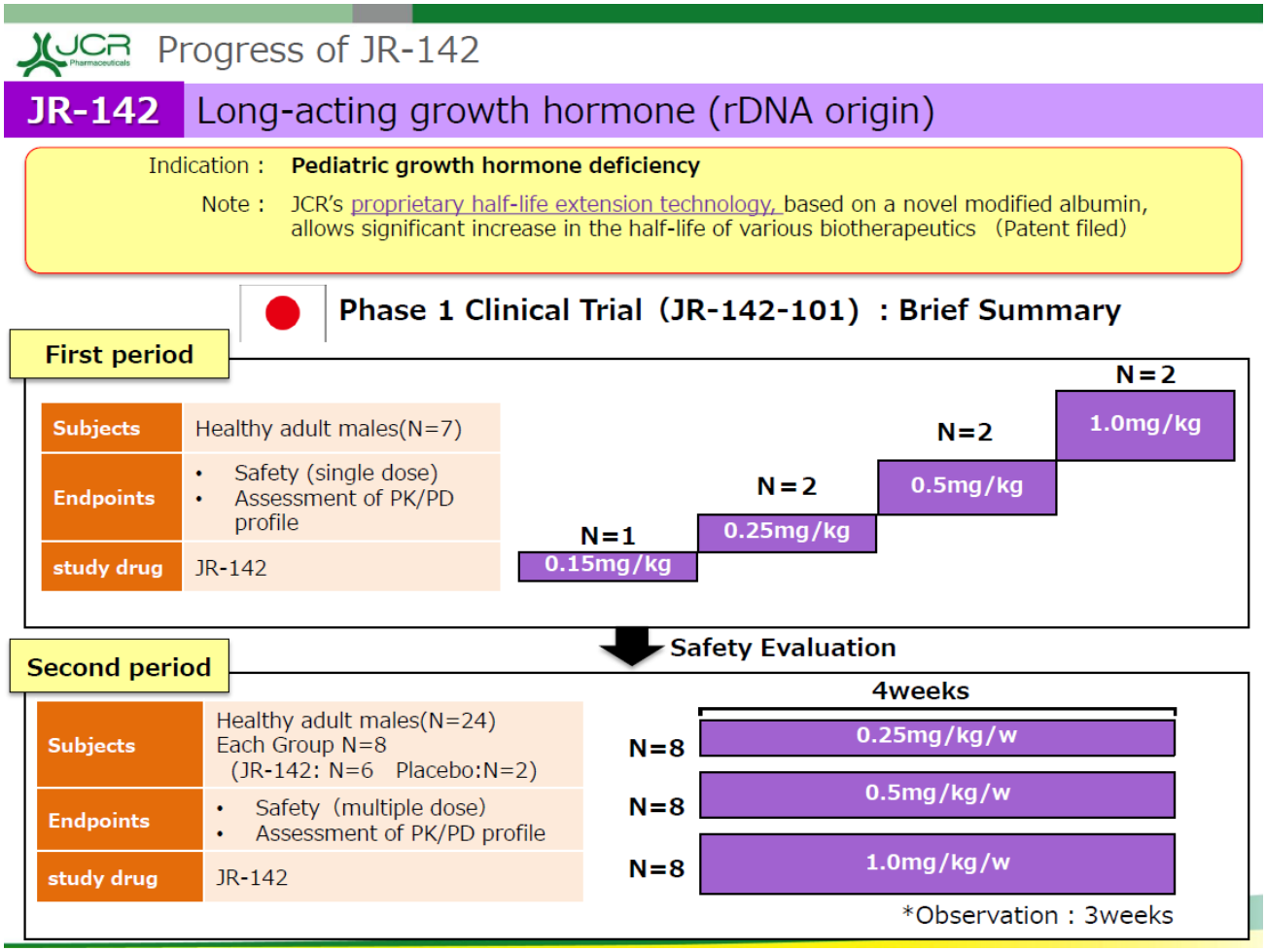
Phase 1/2 Global Clinical Trial (JR-171-101) : Results **Part 1(N=4)**



As you can see here, a decrease in heparan sulfate was confirmed in all cases under the 4-week study.

Taking the average of these results, we have confirmed a reduction of more than 60%, which is the result we had initially expected from this test.

Therefore, I think it will be necessary to examine changes in heparan sulfate over a longer period of time and to study its clinical efficacy in Part 2.



Next is JR-142. I would like to explain the progress in the development of this long-acting growth hormone.

This drug is being developed for the indication of pediatric growth hormone insufficiency-induced short stature. The technology is based on a proprietary modified-albumin, which was developed in-house.

Phase I trials in healthy adults have been completed, and I would like to talk about the results.

Phase I trials are designed to look at safety and PK/PD profiles, so there are 2 parts: a part in which we increase the dose like this in Phase I, and a part in which we administer the drug continuously for 4 weeks.

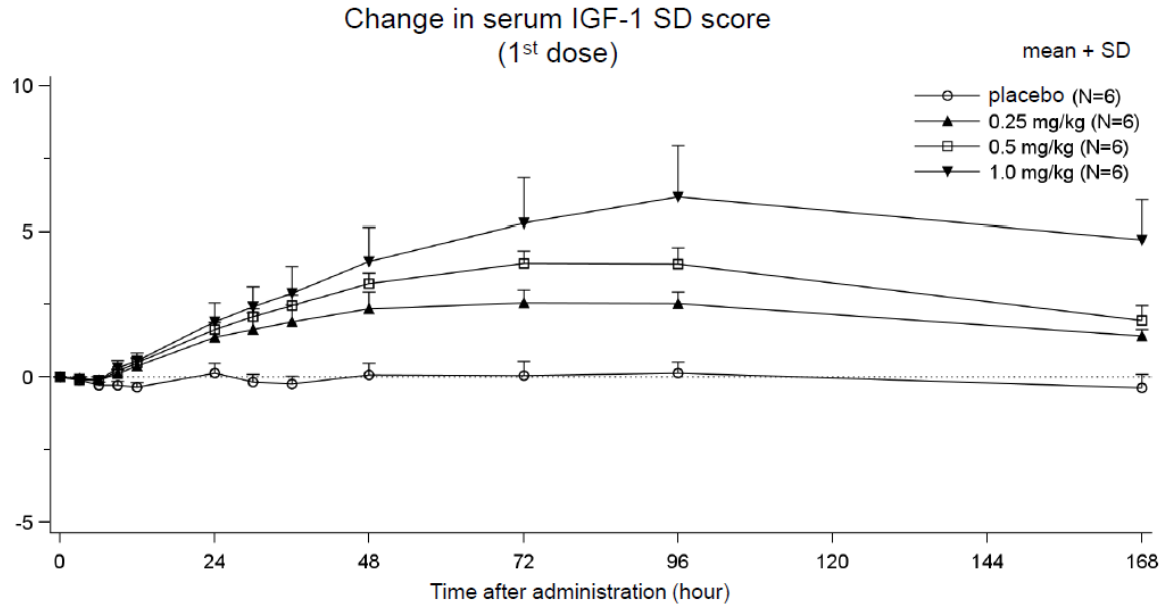
One of the most important points in growth hormone therapy is the increase in the biomarker IGF-1. It is known that increases in IGF-1 contribute to growth.

However, the most important thing is not to raise IGF-1, but rather to ensure that you do not raise it too much. Therefore, as a treatment strategy, it is very important to aim for a narrow concentration range: not too high, not too low.

Progress of JR-142

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

Phase 1 Clinical Trial (JR-142-101): Results



- Dose-dependent increases in PK parameters and pharmacodynamic markers were observed.
- No safety concerns were observed.

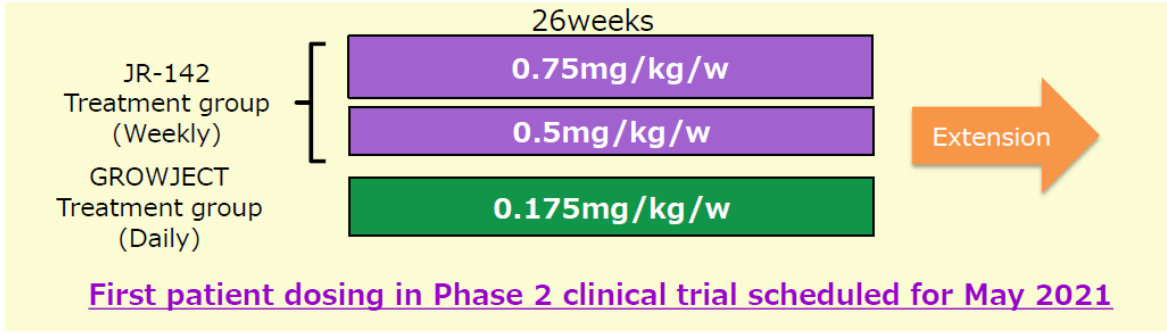
As you can see from this data, there was a very nice dose-dependent increase in IGF-1, and although we are looking at the results for one week, we think we have obtained enough data to consider the dosage for the next study.

Based on these results, we are now preparing for a Phase II study in patients.

Progress of JR-142

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

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



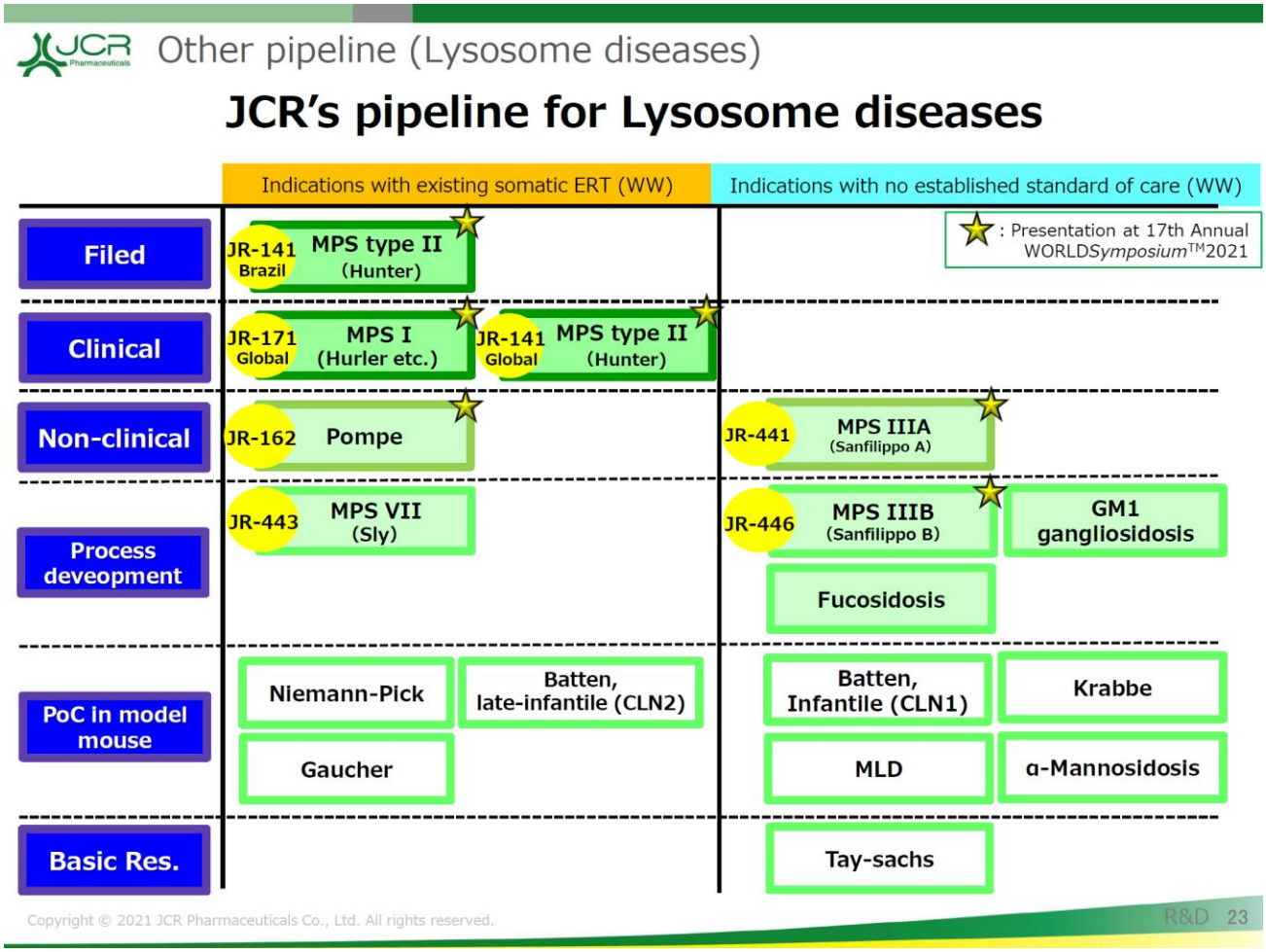
First patient dosing in Phase 2 clinical trial scheduled for May 2021

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
Details	jRCT(Identifier : jRCT2031200372)

This is the outline of the Phase II trial that we are preparing for.

As the target group, we set up daily administration of Growject as the control group, and for the long-acting treatment, we set up 2 groups: a low-dose group and a high-dose group. We are planning to start administration this month. The evaluation item is height.

Medical professionals have high expectations for this drug. We are planning to recruit 24 people under the current arrangement.



Next, I would like to talk about the development of therapeutic agents for other lysosomal diseases.

JR-141 and JR-171 are progressing smoothly in the clinical stage. Some items for tomorrow are JR-162 and JR-441. The latter is a drug for type III mucopolysaccharidosis. In addition, we have other items such as mucopolysaccharidosis type VII and type III compounds in the pipeline. We are keen to move forward with development as soon as possible, given the establishment of the J-Brain Cargo technology in JR-141.

Other pipeline (Lysosome diseases)

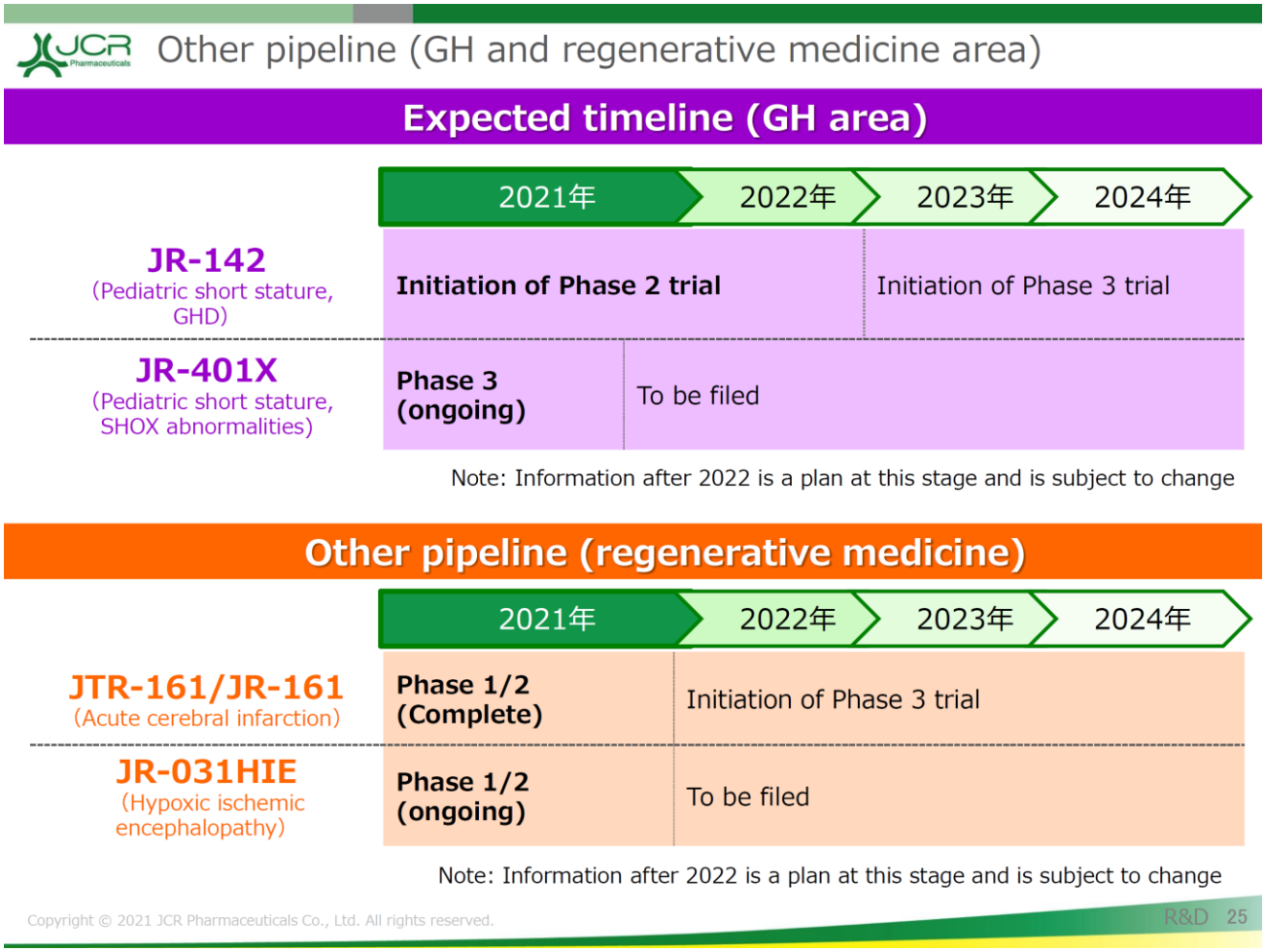
Expected timeline (Lysosome diseases)

	2021	2022	2023	2024
JR-141 pabinafusp alfa (MPS II)	**Japan : Approval** Brazil : To be approved Global : Initiation of Phase 3 trial			
JR-171 (MPS I)	**Global : Phase 1/2 trial (ongoing)**		Initiation of Phase 3 trial	
JR-441 (MPS IIIA)	**Non-clinical (ongoing)**	Initiation of Phase 1/2 trial		
JR-162 (Pompe)	**Non-clinical (ongoing)**		Initiation of Phase 1/2 trial	
JR-443 (MPS VII)	**Non-clinical (ongoing)**		Initiation of Phase 1/2 trial	
JR-446 (MPS IIIB)	**Non-clinical (ongoing)**		Initiation of Phase 1/2 trial	

Note: Information after 2022 is a plan at this stage and is subject to change

You can find the estimated timeline here.

There is no change. We are aiming to start Phase I/II trials for JR-441 next year. We are also planning to start clinical trials for JR-162, JR-443 and JR-446 in 2023.



As I mentioned earlier, JR-142 is now in Phase II trials related to growth hormone.

As for JR-401X, which is a project to expand the indications of Growject for the treatment of SHOX abnormalities, we have already completed recruitment for the Phase III trials and are now observing phase.

As for other regenerative medicine products, JTR-161 will complete Phase I/II clinical trials this year. As for JR-031HIE, we plan to complete Phase I/II trials and consult with the regulatory authorities on the filing of the application in the future.

That concludes my presentation. Thank you very much.

[END]

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