# JCR Pharmaceuticals Co., Ltd.

Financial Results Briefing for the Fiscal Year Ended March 2020 (presentation)

May 18, 2020

# Event Summary

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| **[Company Name]** | JCR Pharmaceuticals Co., Ltd. |
| **[Event Type]** | Earnings Announcement |
| **[Event Name]** | Financial Results Briefing for the Fiscal Year Ended March 2020 (presentation) |
| **[Fiscal Period]** | FY2019 Annual |
| **[Date]** | May 18, 2020 |
| **[Number of Pages]** | 58 |
| **[Time]** | 13:00 – 13:57<br>(Total: 57 minutes, Presentation: 57 minutes, Q&A: 0 minutes) |
| **[Venue]** | Webcast |
| **[Venue Size]** | |
| **[Participants]** | |
| **[Number of Speakers]** | 5 |

| | |
|---|---|
| Shin Ashida | Representative Director, Chairman, President, and CEO |
| Akihiro Haguchi | Executive Director, Administration Division |
| Kazunori Tanizawa | Executive Director, Development Division |
| Hiroyuki Sonoda, PhD | Executive Director, Research Planning Division |
| Toru Ashida | Senior Executive Director, Executive Director, Sales Division, in Charge of Corporate Strategy |

# Presentation

**Shin Ashida:** I will now commence the financial results briefing for the year ended March 31, 2020 for JCR Pharmaceuticals Co., Ltd.

I would like to thank all of our investors for their continued support. Today's presentation will include briefings on the financial results for the year ending March 2020, the status of R&D, and the new medium-term management plan, "Revolution."

I would like to start by talking about the acquisition of ArmaGen, Inc. in the United States.

ArmaGen was founded by Dr. William Pardridge, a former UCLA professor who has been a pioneer in the field of blood–brain barrier transit techniques for more than 50 years. The Company possesses technologies that use anti-insulin receptors and anti-transferrin receptors and has filed more than 50 patents related to blood–brain barrier transit technologies worldwide. Through the acquisition of ArmaGen, we acquired technical assets, including intellectual property rights, which we believe will be very valuable for us in terms of future globalization.

The acquisition of ArmaGen, Inc. was completed on April 10, and we believe that negotiations with other parties will accelerate dramatically in the future with regard to the research of lysosomal disease drugs. The 52-week study of JR-141 has been completed in Japan. The final treatment has been completed in Brazil. In Japan, we have submitted all documents related to the pre-evaluation system based on the preliminary examination and designation system. We plan to submit an application for manufacturing and marketing approval by the end of September. If progress is smooth, we expect to obtain approval during the current fiscal year. We are also planning to launch clinical trials globally for JR-141 and JR-171 this year.

The results of the five-year, medium-term plan, which was announced in 2015, were targeted at sales of JPY25 billion and operating income of JPY5 billion. However, sales of existing mainstay products remained steady, despite the fact that ArmaGen's acquisition procedures resulted in delays in licensing contracts.

As we continue to develop more than 10 drug candidates for lysosomal diseases, clinical trials of JR-141 in Japan and Brazil have clearly demonstrated the efficacy and safety profiles of the drug in humans. In the past five years, we have been able to develop a solid understanding of and confidence in the more than 10 drug candidates being researched for lysosomal disease.

In partnership with Teijin Pharma Limited, there has been good progress in clinical development of regenerative medicine and associated products using dental pulp–derived stem cells.

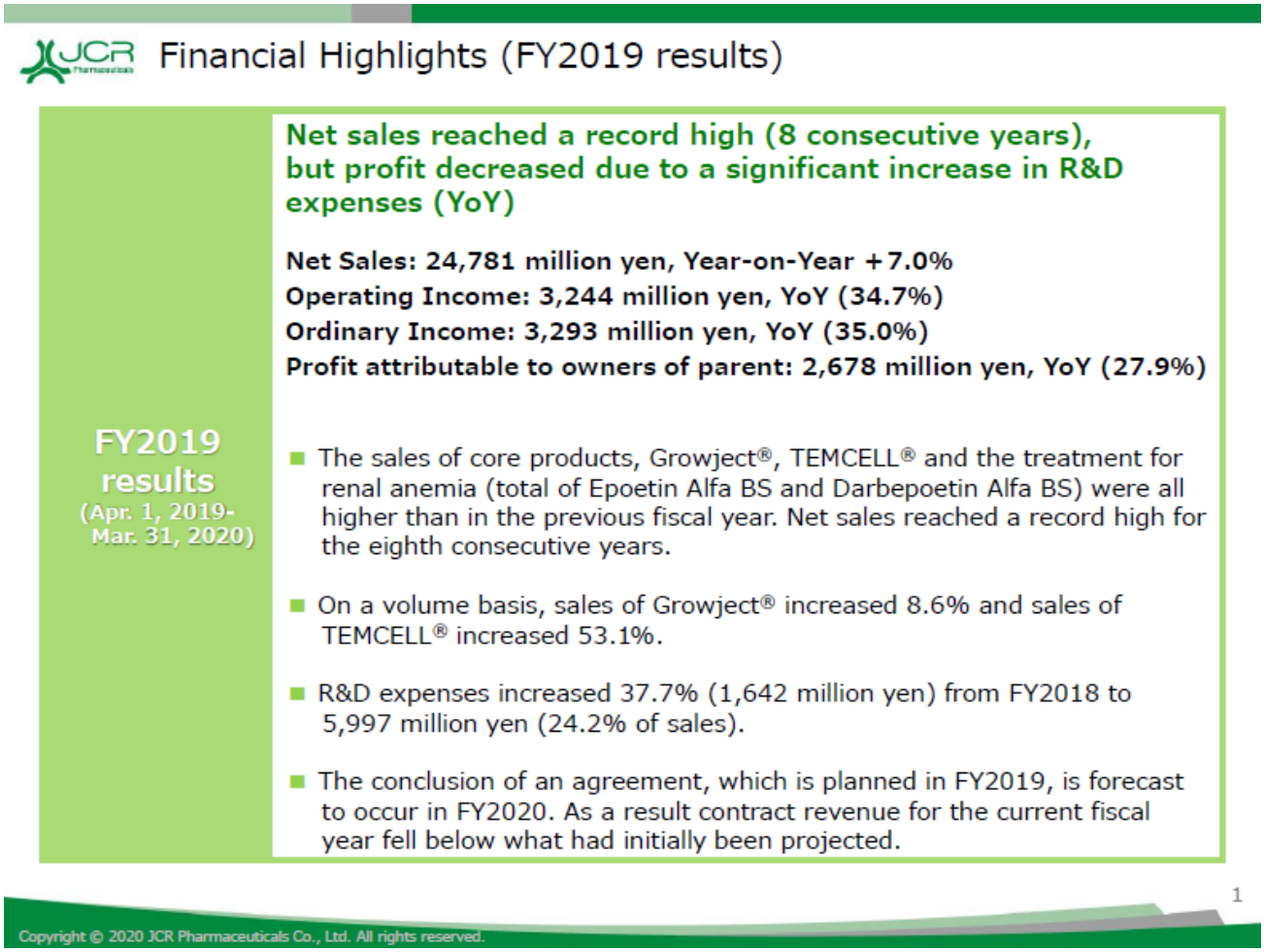
In addition, in the area of research and production systems, we are enhancing research facilities and human resources. We are also expanding our production facilities for investigational drugs and active pharmaceutical ingredients.

Based on these results, we are confident of JCR's future growth and have formulated a new, medium-term, management plan for 2020, "Revolution."

In addition, we will elect two new candidates for the Board of Directors at the General Meeting of Shareholders to be held next month. One is Mathias Schmidt, CEO of ArmaGen. Mr. Schmidt's research track record, as well as his R&D and global clinical-development experience at major pharmaceutical companies, will greatly contribute to our Company. The other is Hiroyuki Sonoda, a very promising young man. We believe that he is capable of identifying the future direction of our R&D and contributing to the development of new

drugs after J-Brain Cargo. I believe that the success of young executives, such as these two individuals, will enable us to achieve significant growth in the future.

We look forward to the continued understanding and support of our investors.




**Haguchi:** I am Akihiro Haguchi, General Manager of the Administration Division. First, we would like to present the financial results for the fiscal year ending March 2020, and then the budget for the fiscal year ending March 2021.

This is a summary of the fiscal year ended March 31, 2020. Sales increased by 7% from the previous period to JPY247 billion, which resulted in an all-product line sales increase, including Growject, Temcell, and products for renal anemia. This has been our eighth year of record-breaking sales. Nevertheless, both operating income and net income declined as R&D expenses rose by JPY1.6 billion compared to the previous fiscal year.

Compared to the forecast at the beginning of the fiscal year, the licensing contracts expected in the fiscal year under review were pushed back to the new fiscal year. This resulted in a decrease in subscription revenue. As a result, both sales and profits decreased compared to the forecast at the beginning of the fiscal year.

## Consolidated Results

(Unit: million yen)

| Consolidated | FY2018 (Apr. 1, 2018- Mar. 31, 2019) A | FY2019 (Apr. 1, 2019- Mar. 31, 2020) B | Year-on-Year (B-A)/A | Reference Initial forecast (before fixing) |
|---|---|---|---|---|
| Net Sales | 23,160 | **24,781** | +7.0% | 26,400 |
| Cost of Sales | 6,567 | **7,901** | +20.3% | 7,300 |
| Gross Profit | 16,592 | **16,880** | +1.7% | 19,100 |
| SG&A | 7,270 | **7,638** | +5.1% | 7,630 |
| R&D Expenses | 4,354 | **5,997** | +37.7% | 6,330 |
| Operating Income | 4,967 | **3,244** | (34.7%) | 5,140 |
| Ordinary Income | 5,068 | **3,293** | (35.0%) | 5,150 |
| Profit attributable to owners of parent | 3,715 | **2,678** | (27.9%) | 4,080 |
| Ratio of Cost of Sales | 28.4% | **31.9%** | +3.5% | 27.7% |
| Ratio of Cost of R&D | 18.8% | **24.2%** | +5.4% | 24.0% |
| Operating Profit Ratio | 21.4% | **13.1%** | (8.3%) | 19.5% |

(Reference)

| | | | | |
|---|---|---|---|---|
| R&D expenses* | 5,270 | **6,521** | +23.7% | 6,921 |

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

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Next, we look at the summary of profit and loss.

As I have explained, sales of our mainstay products increased 7% YoY, but R&D expenses increased significantly, resulting in a decrease in operating income and other income.

The ratio of cost of sales to net sales increased by 3.5% to 31.9%, mainly due to a decline in the ratio of contract revenue from 15% to 8%.

The R&D ratio rose 5.4% to 24.2%, and the operating margin decreased 8.3% to 13.1%.

## Sales by Business Segments (Consolidated)

(Unit: million yen)

| Name of business segment | FY2018 (Apr. 1, 2018- Mar. 31, 2019) | | FY2019 (Apr. 1, 2019- Mar. 31, 2020) | | |
|---|---|---|---|---|---|
| | A | Composition ratio | B | Composition ratio | Year-on-Year (B-A)/A |
| Growject® | 11,978 | 51.7% | **12,650** | 51.0% | +5.6% |
| Epoetin Alpha BS Inj.[JCR] | 4,511 | 19.5% | **4,097** | 16.5% | (9.2%) |
| Darbepoetin Alpha BS Inj.[JCR] | — | — | **1,412** | 5.7% | — |
| TEMCELL®HS Inj. | 2,041 | 8.8% | **3,126** | 12.6% | +53.2% |
| Agalsidase Beta BS I.V. Infusion [JCR] | 74 | 0.3% | **317** | 1.3% | +327.4% |
| Urine-derived products | 690 | 3.0% | **1,041** | 4.2% | +50.8% |
| License Revenue | 3,560 | 15.4% | **2,050** | 8.3% | (42.4%) |
| Other | 303 | 1.3% | **84** | 0.3% | (72.0%) |
| **Total Net Sales** | **23,160** | **100.0%** | **24,781** | **100.0%** | **+7.0%** |

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The breakdown of net sales can be found in the following section.

Sales of Growject rose 5.6%, and sales of drugs for renal anemia, including darbepoetin alfa and epoetin alfa BS, rose by approximately JPY1 billion, or 22%. Sales of Temcell increased by 53%. Sales of agalsidase beta and urine-derived products also increased. This means that there was growth in all product groups.

Although contract revenues declined, all product lines saw higher revenues, resulting in a 7% YoY increase in total revenues.

## Financial Statement (Consolidated)

(Unit: million yen)

| | Mar. 2019 | **Mar. 2020** | Main increase/decrease | | Mar. 2019 | **Mar. 2020** | Main increase/decrease |
|---|---|---|---|---|---|---|---|
| **Current assets** | 27,368 | **28,342** | Cash and deposit +3,136<br>Accounts receivable △857<br>Securities △441 | **Current liabilities** | 8,684 | **10,434** | Short-term loans payable +1,250<br>Account payable +356 |
| | | | | **Non-current liabilities** | 2,957 | **4,761** | Long-term loans payable +1,950 |
| **Non-current assets** | 15,147 | **19,433** | Property, plant and equipment +3,813 | **Total liabilities** | 11,642 | **15,195** | +3,553 |
| | | | | **Total net assets** | 30,874 | **32,579** | Profit +1,689 |
| **Total** | 42,516 | **47,775** | +5,259 | **Total** | 42,516 | **47,775** | +5,259 |

| | | | | | | |
|---|---|---|---|---|---|---|
| **Capital investment** | 1,517 | **5,296** | | **Equity ratio** | 71.1% | **66.6%** |

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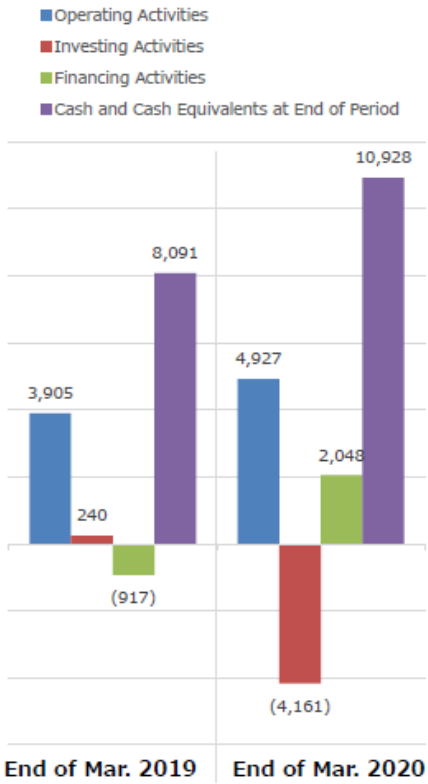
In the Assets section, property, plant, and equipment increased by JPY3.8 billion, and in the Liabilities section, long- and short-term borrowings increased by JPY3.2 billion. This was due to acquisition of land and buildings for construction of the Second Investigational Drug Manufacturing Center, and to increase the number of research facilities. This was funded through borrowings.

Capital expenditures for the fiscal year under review were approximately JPY5.3 billion. Of this, approximately JPY4 billion is attributable to the acquisition of land and buildings for the purpose of constructing the Second Investigational Drug Manufacturing Center and expanding research facilities.

## Cash Flows (Consolidated)

(Unit: million yen)




| | End of Mar. 2019 A | End of Mar. 2020 B | Increase/ decrease B-A |
|---|---|---|---|
| Income before income taxes | 4,928 | **3,422** | (1,506) |
| Depreciation and amortization | 1,343 | **1,434** | +91 |
| Accounts receivable-trade | (1,732) | **857** | +2,589 |
| Inventories | (157) | **258** | +415 |
| Other | (476) | **(1,045)** | (569) |
| **Operating Activities** | 3,905 | **4,927** | +1,022 |
| Securities | 1,256 | **668** | (648) |
| Capital investment | (898) | **(4,838)** | (3,943) |
| Other | (117) | **8** | +125 |
| **Investing Activities** | 240 | **(4,161)** | (4,401) |
| Loans payable | 86 | **3,200** | +3,114 |
| Dividends・treasury stock | (807) | **(973)** | (166) |
| Other | (196) | **(177)** | (1) |
| **Financing Activities** | (917) | **2,048** | +2,965 |
| **Cash and Cash Equivalents at End of Period** | 8,091 | **10,928** | +2,837 |

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Net cash provided by operating activities was JPY4.9 billion. Net cash outflow from investing activities was JPY4.1 billion for capital expenditures, and net cash provided by financing activities was JPY2 billion from borrowings. Cash equivalents at the end of the fiscal year were JPY10.9 billion.

The above is a summary of the financial results for the year ended March 31, 2020.

## Forecast Highlights (FY 2020)

**Earnings Forecast**
**(Apr. 1, 2020- Mar. 31, 2021)**

**Net Sales: 27,200 million yen, Year-on-Year + 9.8%**
**Operating Income: 6,000 million yen, YoY +84.9%**
**Ordinary Income: 6,000 million yen, YoY +82.2%**
**Profit attributable to owners of parent: 4,800 million yen, YoY +79.2%**

- The sales of Growject®, treatment renal anemia (total of Epoetin Alfa BS, Darbepoetin Alfa BS) and Agalsidase beta BS are expected to increase.
- Revenue in licensing is expected to Increase, including the revenue from contract that fell short of the previous fiscal year.
- Based on this factors, Operating income is forecast to increase by 84.9% after absorbing increase in R&D expenses.

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Next, we look at the forecasts for the fiscal year ending March 2021.

In terms of sales, we anticipate an increase in revenue from Growject, renal anemia drugs, and agalsidase beta BS. We anticipate an increase in contract income of approximately JPY42 billion, which includes the amount carried forward from the previous period.

As a result, net sales are forecast to increase 9.8% YoY to JPY27.2 billion. On the other hand, although R&D expenses are expected to increase further, we expect to absorb this increase and forecast an increase in both operating income and net income of approximately 80%.

## FY2020 Forecast (Consolidated)

(Unit: million yen)

| Consolidated | FY2019 (Apr. 1, 2019- Mar. 31 2020) A | **FY2020 (Apr. 1, 2020- Mar. 31, 2021) B** | Increase/ decrease B - A | Year-on-Year (B-A)/A |
|---|---|---|---|---|
| **Net sales** | 24,781 | **27,200** | +2,418 | +9.8% |
| **Cost of sales** | 7,901 | **6,800** | (1,101) | (13.9%) |
| **Gross profit** | 16,880 | **20,400** | +3,519 | +20.9% |
| **SG&A** | 7,638 | **8,000** | +361 | +4.7% |
| **R&D Expenses** | 5,997 | **6,400** | +402 | +6.7% |
| **Operating Income** | 3,244 | **6,000** | +2,755 | +84.9% |
| **Ordinary Income** | 3,293 | **6,000** | +2,706 | +82.2% |
| **Profit attributable to owners of parent** | 2,678 | **4,800** | +2,121 | +79.2% |

| | | | | |
|---|---|---|---|---|
| **Ratio of Cost of Sales** | 31.9% | **25.0%** | (6.9%) | |
| **Ratio of Cost of R&D** | 24.2% | **23.5%** | (0.7%) | |
| **Operating Profit Ratio** | 13.1% | **22.1%** | +9.0% | |
| (Reference) | | | | |
| **R&D expenses*** | 6,521 | **7,600** | +1,078 | +16.5% |

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

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Next, I have outlined our earnings forecast for the fiscal year ending March 2021.

We forecast a 9.8% increase in net sales and an 80% increase in profit. On this page, I would like to point out that, despite the increase in sales, the cost of sales is expected to decline.

One factor behind this is that sales of urine-derived products, which were at around JPY1 billion in the previous fiscal year, will not be present. Also, sales of epoetin alpha BS are expected to decline. However, sales of agalsidase beta BS are expected to increase by approximately the same amount. The difference in profitability between epoetin alfa BS and agalsidase beta BS is one factor.

## FY2020 Forecast (Consolidated)

(Unit: million yen)

| Name of Business segment | FY2019 (Apr. 1, 2019-Mar. 31 2020) A | Composition ratio | FY2020 forecast (Apr. 1, 2020- Mar. 31, 2021) B | Composition ratio | Increase/ decrease (B-A) |
|---|---|---|---|---|---|
| Growject® | 12,650 | 51.0% | 13,270 | 48.8% | +620 |
| Treatment for renal anemia | 5,509 | 22.2% | 6,540 | 24.0% | +1,031 |
| Epoetin Alpha BS Inj. [JCR] | 4,097 | 16.5% | 3,350 | 12.3% | (747) |
| Darbepoetin Alfa BS Inj. [JCR] | 1,412 | 5.7% | 3,190 | 11.7% | +1,778 |
| TEMCELL®HS Inj. | 3,126 | 12.6% | 2,110 | 7.8% | (1,016) |
| Agalsidase Beta BS I.V. Infusion [JCR] | 317 | 1.3% | 1,060 | 3.9% | +743 |
| License Revenue | 2,050 | 8.3% | 4,220 | 15.5% | +2,170 |
| Other | 1,125 | 4.6% | 0 | 0.0% | (1,125) |
| **Total Net Sales** | **24,781** | **100.0%** | **27,200** | **100.0%** | +2,419 |

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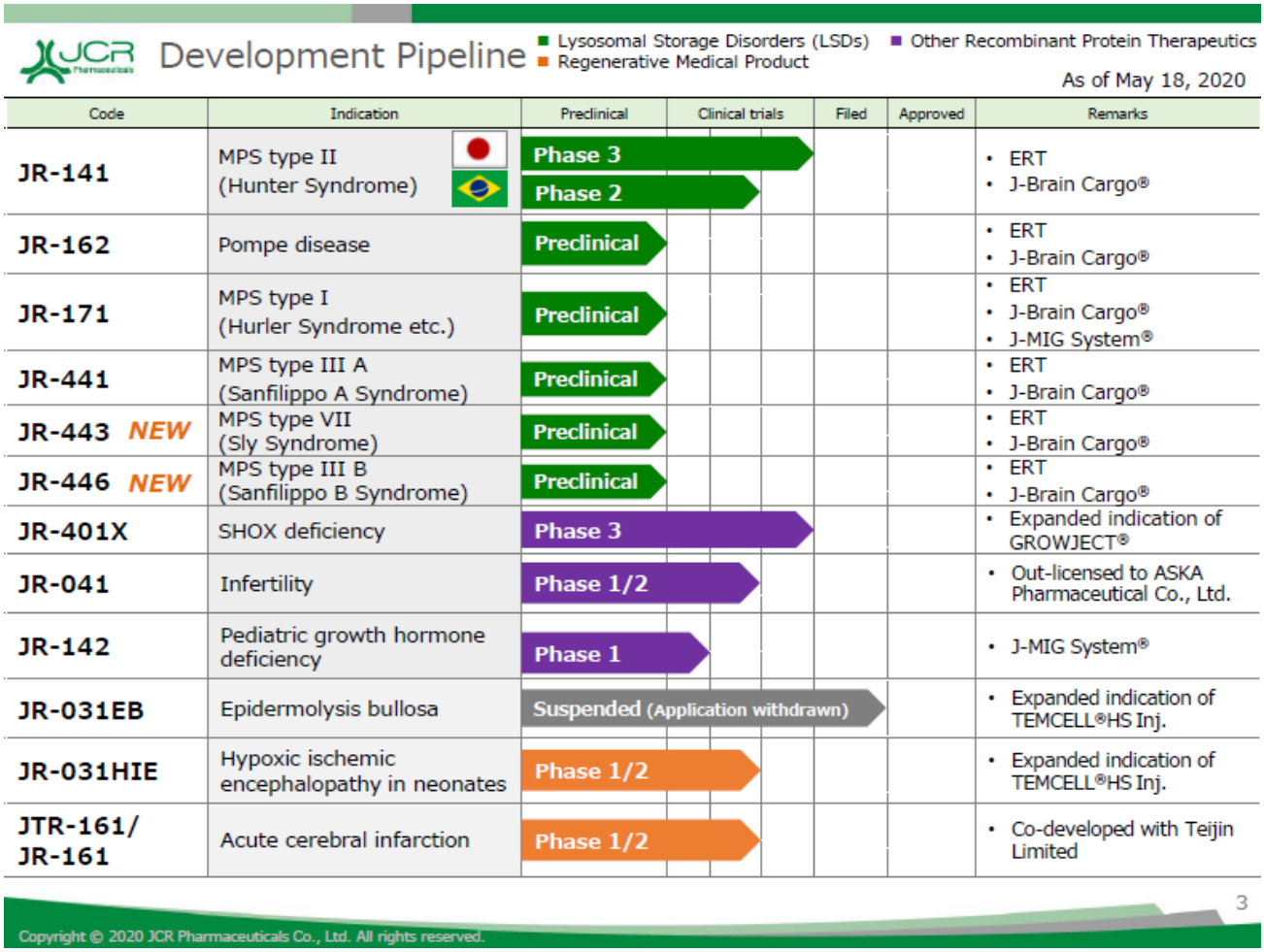
Finally, we look at the breakdown of our sales forecasts for the year ending March 2021.

In addition to Growject, treatments for renal anemia, and agalsidase beta BS, we anticipate an increase in contract income. On the other hand, we expect a decline in revenue for the new fiscal year. Sales of other products are expected to decline by about JPY1.1 billion. This is due to the absence of urine-derived products, as I mentioned earlier.

As a result, net sales are forecast to increase by 9.8% compared to the current fiscal year.

This concludes the summary of the fiscal year ending in March 2020 and the forecast for March 2021. Thank you very much for your attention.



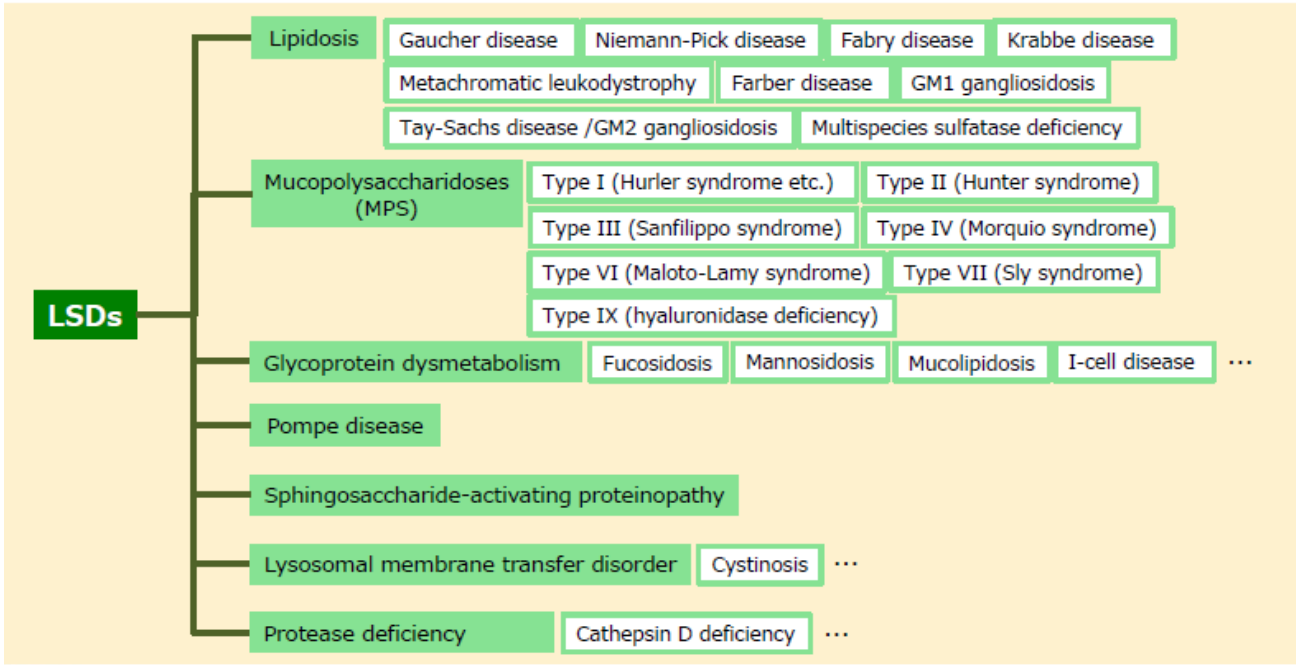

**Tanizawa:** I would like to report on the progress of the development items.

The development pipeline will be shown here as well. The green area is treatments for lysosomal disease. Two new products have been added this time. The purple portion includes growth hormone–based treatments and protein formulations, and the orange portion denotes regenerative medicine and other products.

Today, we have compiled 52 weeks of domestic market data for JR-141, so I will report on that first. I will then explain about the other products under development.

## Lysosomal Storage disorders (LSDs)

LSD is a group of rare inherited disorders in which one of enzymes in the lysosomes is congenitally missing or functionally deficient, resulting in the accumulation of metabolic wastes which fail to dissolve.
Their symptoms vary depending on the affected enzymes and the accumulating substrates.
They are designated by MHLW as intractable disease as well as specific pediatric chronic disease.





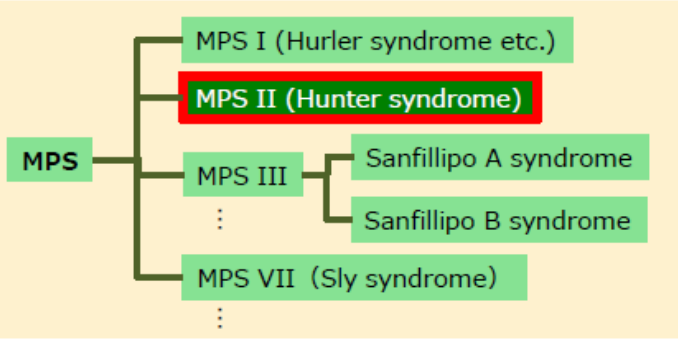

We are focusing our efforts on lysosomal disease. These are congenital conditions, where the function of the enzymes within lysosomes is reduced. As a result, the products that these enzymes are supposed to break down end up accumulating in the body. Lysosomal diseases can be categorized by the substances that accumulate, such as fats, mucopolysaccharides, and so on.

## Progress of JR-141

**JR-141** BBB-penetrating iduronate-2-sulfatase (rDNA origin)

BBB : Blood Brain Barrier

| | |
|---|---|
| Indication | **MPS type II (Hunter syndrome)** |
| Patient population*1 | 250 (Japan) , 7,800 (WW) est. |
| Market size*2 | 7.6 billion JPY est. (2019 Japan), 87 billion JPY est. (2019 WW) |
| Disease overview | A X-linked recessive disease caused by a deficiency of the enzyme iduronate-2-sulfatase that metabolizes mucopolysaccharides within the body. Symptoms are systemic and multiple; **central nervous system (CNS) disorders** is notable in particular.. |




*1 Calculated internally based on the date from MHLW *2 Actual sales of existing ERT and data from Evaluate Pharma and IQVIA

**Existing enzyme replacement therapy does not show effects on CNS symptoms due to non-penetration of BBB**

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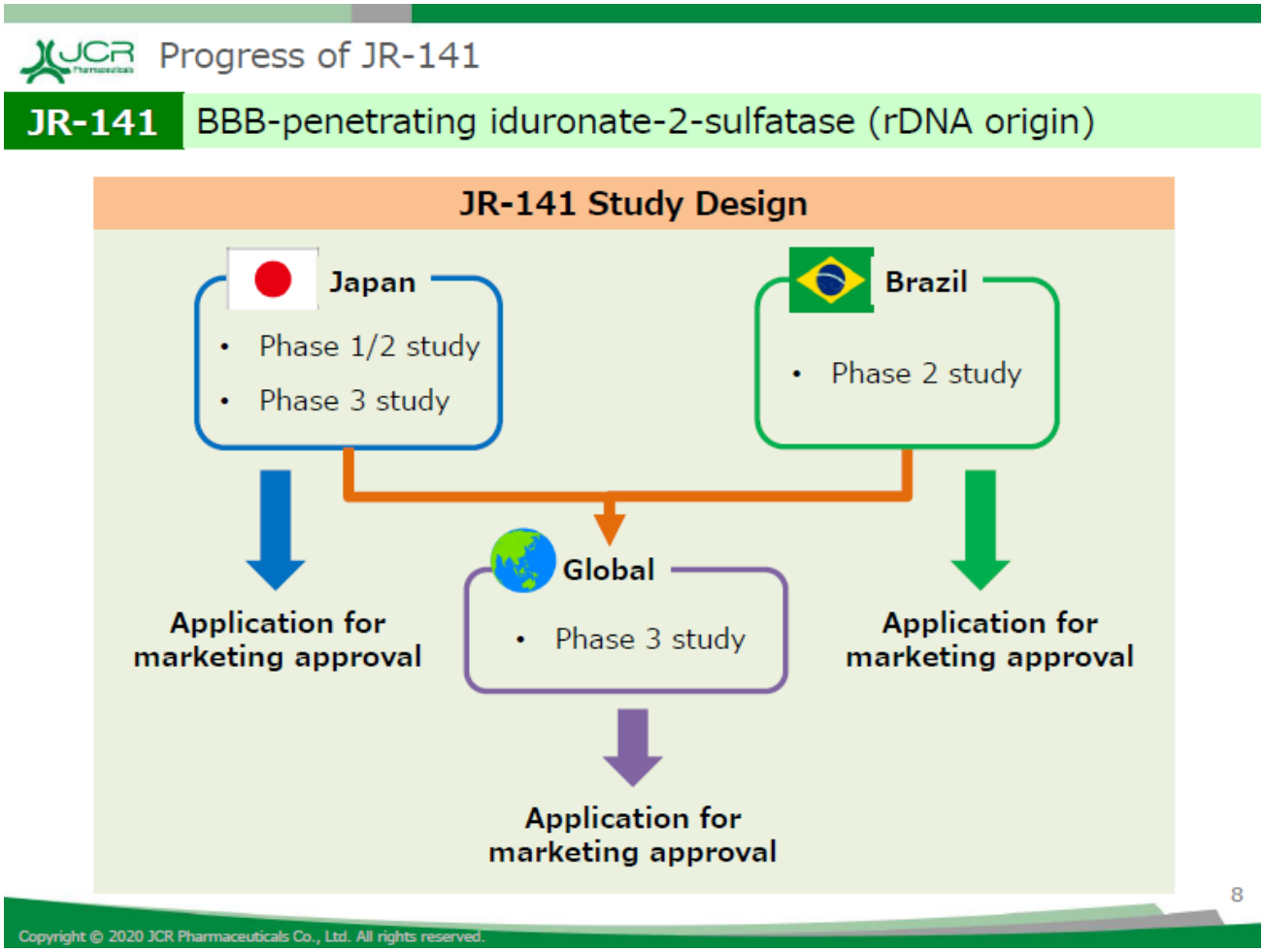
In particular, JR-141 is a treatment for Hunter Syndrome, a condition resulting from build-up of mucopolysaccharides. The number of patients is estimated to be 250 in Japan and 7,800 overseas. Although we have an existing enzymatic formulation, this cannot readily pass through the blood–brain barrier, resulting in limited effectiveness in the central nervous system.

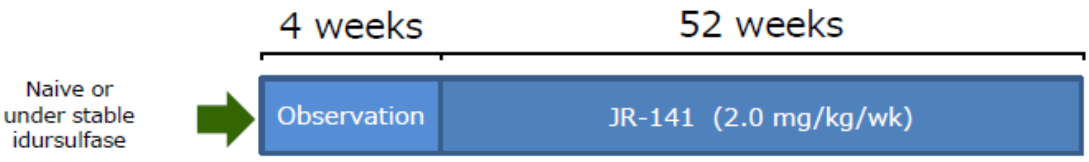



JR-141 is already being developed. The Phase 3 trial in Japan has been completed. In Brazil, Phase 2 testing, to look at administration and doses, has also been completed. Based on these data, we will submit an application for marketing approval this year. In parallel, we are planning a global, clinical, Phase 3 study in the US, Europe, and Brazil.

## Progress of JR-141

**JR-141** BBB-penetrating iduronate-2-sulfatase (rDNA origin)

### Phase 3 study (JR-141-301): Study design




| | |
|---|---|
| Primary Endpoint | Change of Heparan Sulfate (HS) in Cerebrospinal Fluid (CSF) |
| Secondary Endpoints | • Neurocognitive test, Adaptive behavioral test<br>• HS and DS in serum and Urine<br>• Liver Volumes, Spleen volumes<br>• 6-minute walk test<br>• Joint range of motion |
| Number of Subjects | 28 |

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Here, I would like to explain the outcomes of clinical trials of JR-141 in Japan.

Regarding this study, eligible patients were those who have been administrated idursulfase or who have not received treatment. JR-141 was subsequently administered at a rate of 2.0 mg/kg/week for 52 weeks.

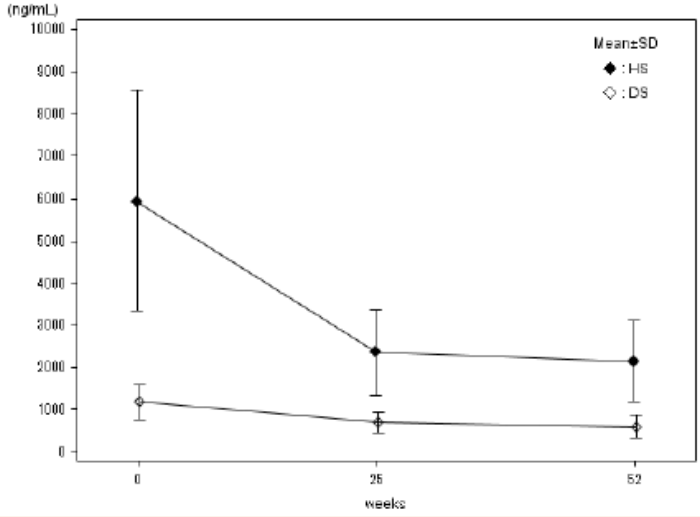
The primary endpoint is the concentration of heparan sulfate in the cerebrospinal fluid (CSF). Secondary endpoints were development and systemic problems. A total of 28 patients from 19 hospitals participated in the trial.

Progress of JR-141

**JR-141** BBB-penetrating iduronate-2-sulfatase (rDNA origin)

**Phase 3 study (JR-141-301): Results (52 weeks)**

<Concentrations of HS and Dermatan Sulfate (DS) in CNS>




HS and DS concentrations in CSF decreased in all subjects
→HS concentrations in CSF decreased significantly (-61.3%±12.1%, p<0.001)
52 weeks average: 2124±882.6 ng/mL

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This indicates the decline in substance concentration in the CSF over time.

To summarize, the primary evaluation endpoint was met. As you can see in the graph above, the concentration of heparan sulfate declined from nearly 6,000 at the start of administration to 2,124 at 52 weeks. There was a decline in CSF heparan sulfate concentration in all subjects. Heparan sulfate is a biomarker whose correlation with central nervous system conditions is well understood in the context of mucopolysaccharidosis. Indeed, our data also show that for severely sick patients, the baseline level of heparan sulfate is higher than that of mild-type patients.

We believe that this decrease in substance concentration in the brain and spinal fluid is important data, suggesting the effect of this drug on central neurological symptoms.

Progress of JR-141

**JR-141** BBB-penetrating iduronate-2-sulfatase (rDNA origin)

## Phase 3 study (JR-141-301): Results (52 weeks)

**<New Kyoto Scale Developmental Assessment of Age Equivalence (AE) : after 52 weeks>**

| Classification of Disease phenotype | Number of subjects | Slope |
|---|---|---|
| Attenuated | 8 | 0.9543 |
| Severe: Initial phase | 2 | 0.6705 |
| Severe: Middle phase | 11 | -0.0802 |
| Severe: Late phase | 5 | -0.0904 |

**Initial phase :**
Age < 3y and developmental index > 80
**Middle phase :**
Age 8y or younger, or developmental index of > 20
**Late phase :**
Age > 8y or developmental index < 20

**Interpretation of the data**

- Attenuated : AE developed almost normally (normal development = slope 1)
- Severe (<3y) : AE improved (neurodegeneration suppressed)
- Severe (>3y) : AE stabilized (deteriorations of neurodegeneration suppressed)

**Therapeutic implications**

- Early intervention maintains CSF substrate concentrations in low level and maintains AE development.
- JR-141 maintains AE by suppressing deteriorations of neurodegeneration even in severe subjects.

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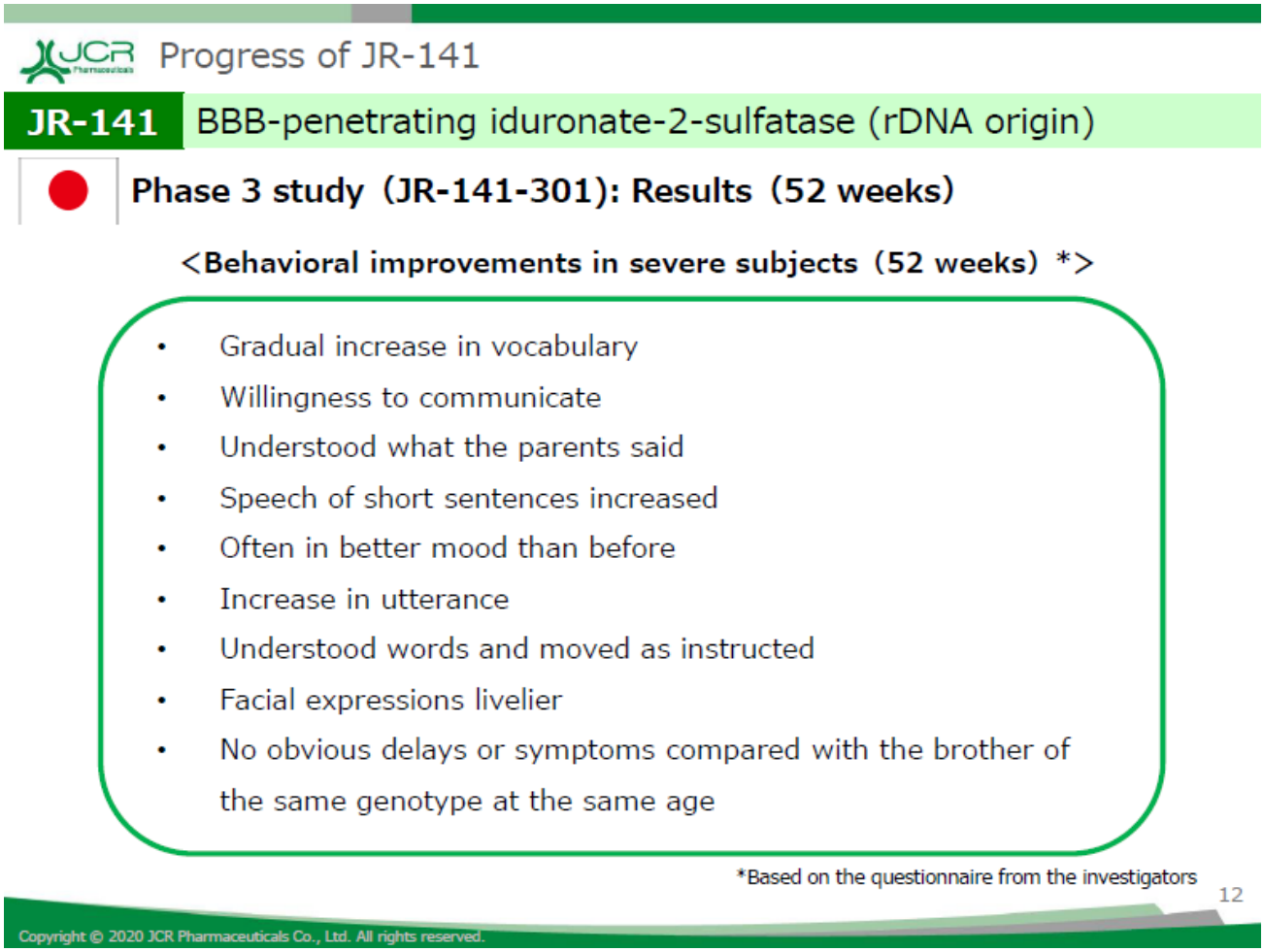
This slide shows changes in developmental age after 52 weeks of treatment. I will add a little explanation to this slide.

The term “attenuated” refers to the mild type. “Severe” is for those with severe disease. Among patients with severe disease, those under the age of three years were categorized as “initial phase” and those over the age of three as ”middle” or “late.” The changes in developmental age of each patient at 52 weeks are described in terms of slope.

Slope is 0.95 for attenuated patients. Slope is 1 when the age of development rises by 12 months after treatment for 12 months, so it can be seen that 0.95 is almost normal development. The slope is 0.67 among patients aged three or under with severe disease. This indicates that the age of development has increased.

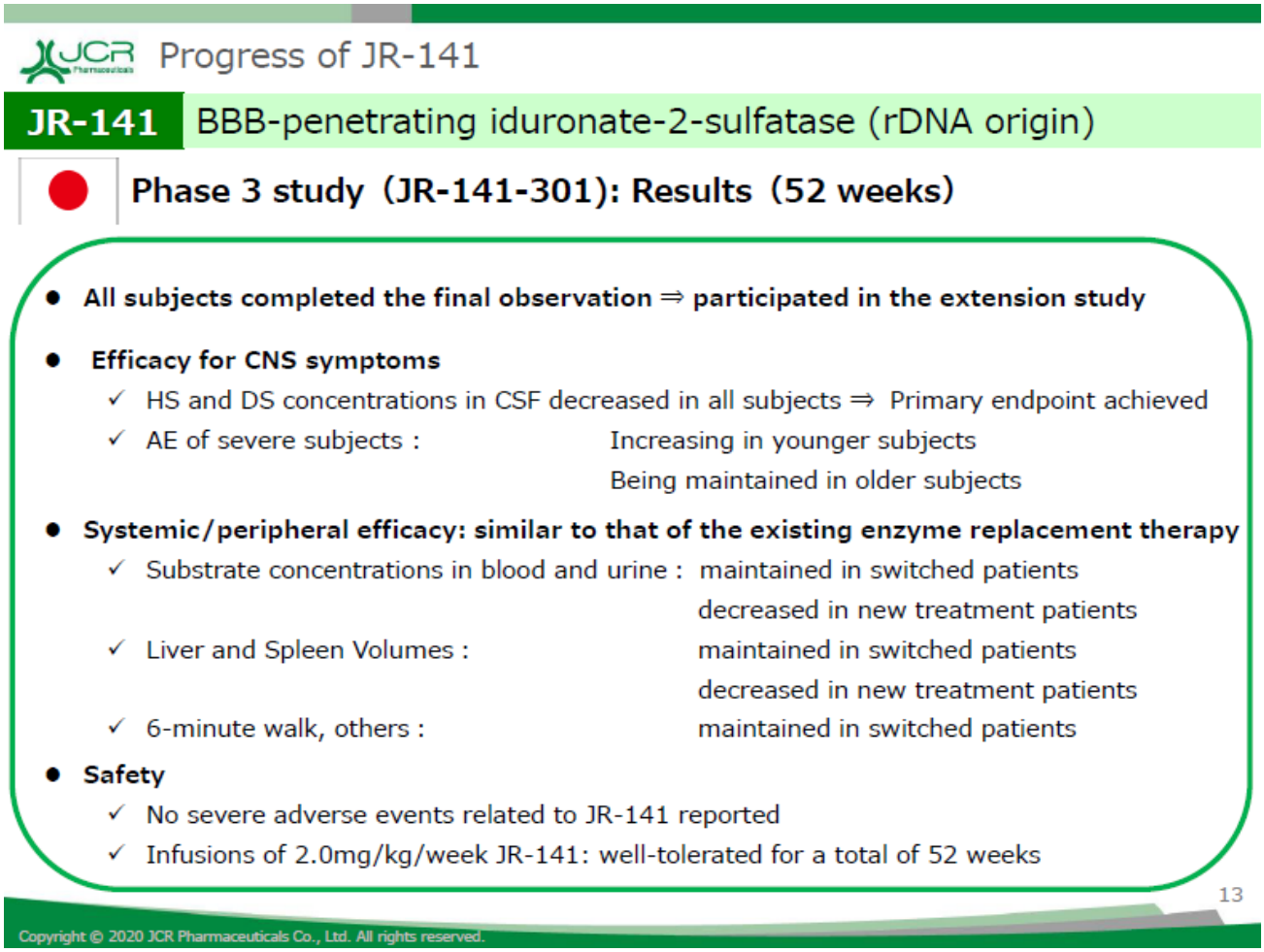
For patients aged three and over, the slope is -0.08 or -0.09, so developmental age is nearly constant. We believe that early treatment has the potential to sustain development by keeping the level of substrate in the brain at a low level. In addition, it has been shown that even in severely impaired patients, it may be possible to reduce the deterioration of neurodegeneration and maintain their developmental age. In the future, we intend to conduct a longer-term evaluation and make comparisons with the natural history of the disease.




Behavioral improvements in patients with severe disease are shown in this slide.

These data are in the form of a case survey form, which is received from the doctors in charge. There have been many reports of improvements in language, liveliness, expression, and recognition, such as gradual increase in vocabulary, willingness to communicate, increased talkativeness, good mood, and increased vocabulary. It is important to note that these changes are noted even in patients with severe disease. Finally, as I will mention, it is extremely important that there are no obvious delays or problems compared with siblings of the same genetic type.

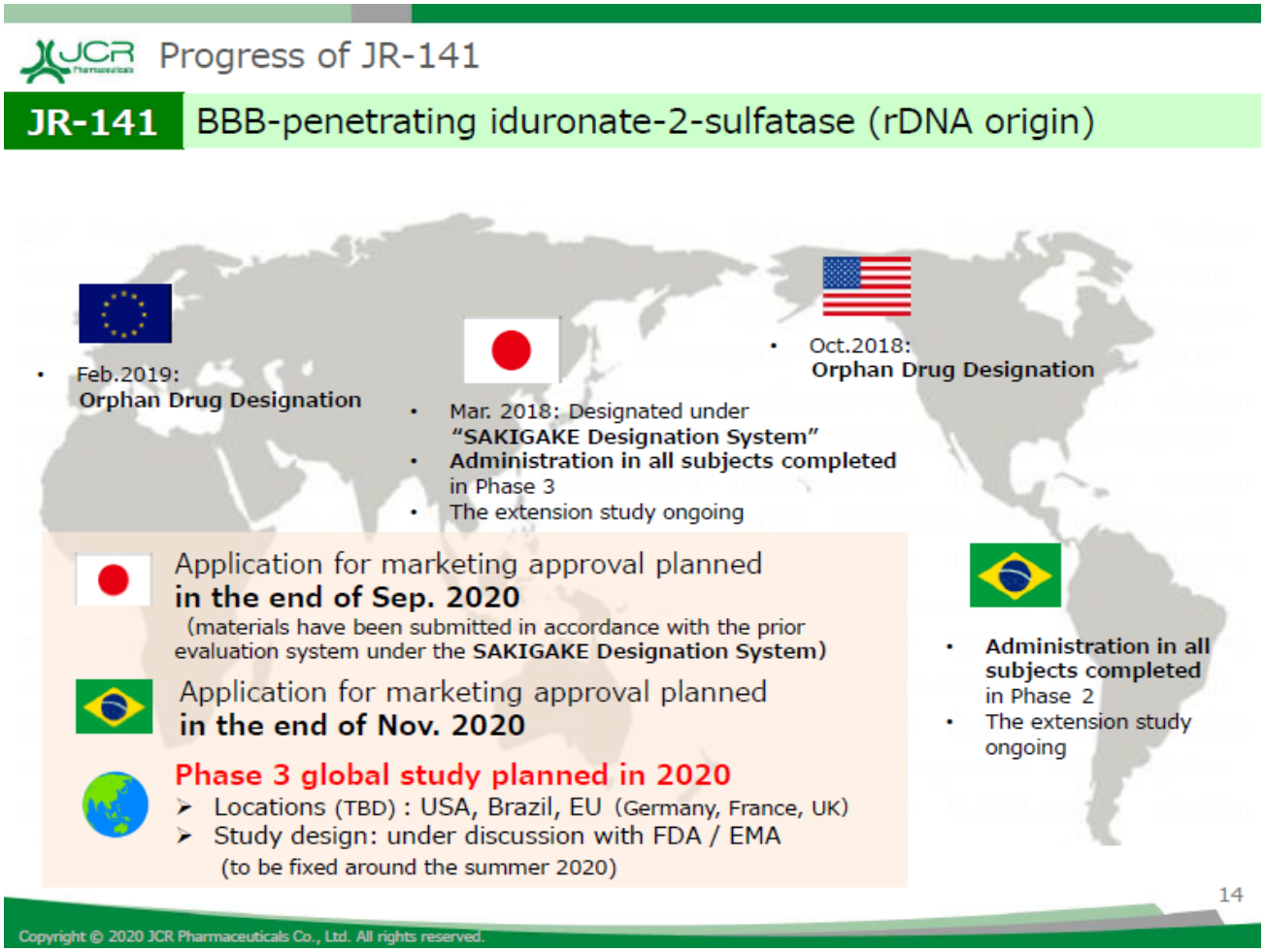
So far, I have reported on the three aspects of biomarker reduction, maintenance, or improvement of development scores, and behavioral improvement. I would also like to emphasize the holistic benefits to patients and their families.




This slide shows a summary of the test results.

With regard to the Phase 3 study in Japan, the final observation has been completed for all cases. For CNS symptoms, the primary endpoint of the study has been met. Regarding developmental age, the figure in young patients tended to increase after treatment, and the figure in older patients was maintained. We have not presented the data here, but the effectiveness for systemic symptoms was about the same as that of existing enzymes in terms of biomarker changes, hepatosplenomegaly, and a six-minute walking test result.

There were no serious adverse events related to the administration of JR-141. We conclude that treatment with 2.0 milligrams of JR-141 was very well tolerated.




Based on these efficacy and safety results, we plan to submit an application for marketing approval in Japan at the end of September 2020. We have already completed the submission of all parts of the advance assessment under the sakigake pioneering treatment designation. In Brazil, an application for manufacturing and marketing approval is scheduled for the end of November 2020. At the same time, we are preparing for global testing in the United States, Brazil, and Europe during 2020. We are currently in the process of discussions with the regulatory authorities in each country.

We recognize that JR-141 has been very successful, so we would like to aggressively develop treatment for other forms of mucopolysaccharidosis.

## Progress of Other Compounds

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

| | |
|---|---|
| Indication | **MPS type I (Hurler syndrome, Hurler-Scheie syndrome, Scheie syndrome)** |
| Patient population*1 | 60 (Japan), 3,600 (WW) est. |
| Market size*2 | 1.6 billion JPY est. (2019 Japan) , 28 billion JPY est. (2019 WW) |
| Disease overview | An autosomal recessive disease caused by a deficiency of the enzyme α-L-iduronidase that metabolizes mucopolysaccharides within the body. Symptoms are systemic and multiple; **CNS disorders** is notable in particular. |

*1 Calculated internally based on the date from MHLW *2 Actual sales of existing ERT and data from Evaluate Pharma and IQVIA

- Phase 1/2 study: Study design (planned)
  - Number of subjects : 19
  - Country : Japan, USA, Brazil
  - Administration period : 12 weeks
  - Primary Endpoint : Safety
  - Secondary Endpoint : effects for CNS symptoms and Systemic symptoms
    Plasma pharmacokinetics

▶ Phase 1/2 study is planned in 2020

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JR-171 is a treatment for type I mucopolysaccharidosis. We are currently holding discussions with regulatory authorities in each country with the aim of starting Phase 1/Phase 2 trials in 2020. We expect the trials to begin as scheduled, as discussions are in the final stage.

## Progress of Other Compounds

### JR-441 BBB-penetrating heparan N-sulfatase (rDNA origin)

| | |
|---|---|
| Indication | **MPS type III A (Sanfilippo A syndrome)** |
| Patient population*1 | 60 (Japan) , 6,900 (WW) est. |
| Market size*2 | No existing drug |
| Disease overview | An autosomal recessive disease caused by a deficiency of the enzyme heparan-N-sulfatase that metabolizes mucopolysaccharides within the body. Notably, rapid progression of **CNS disorders** affects neurocognitive development, with a peak at 2 or 3 years of age. Type III A is relatively severe. Hematopoietic stem cell transplantation can be a treatment option, but its effectiveness remains to be established. |

*1 Calculated internally based on the date from MHLW (Total of Type A&B) *2 Actual sales of existing ERT and data from Evaluate Pharma and IQVIA

- Feb. 2020: New guidance about developing drugs for treatment of MPSIII issued from the FDA

Overall JR-441 development strategy to be reviewed accordingly

- Phase 1/2 study is planned in FY 2022

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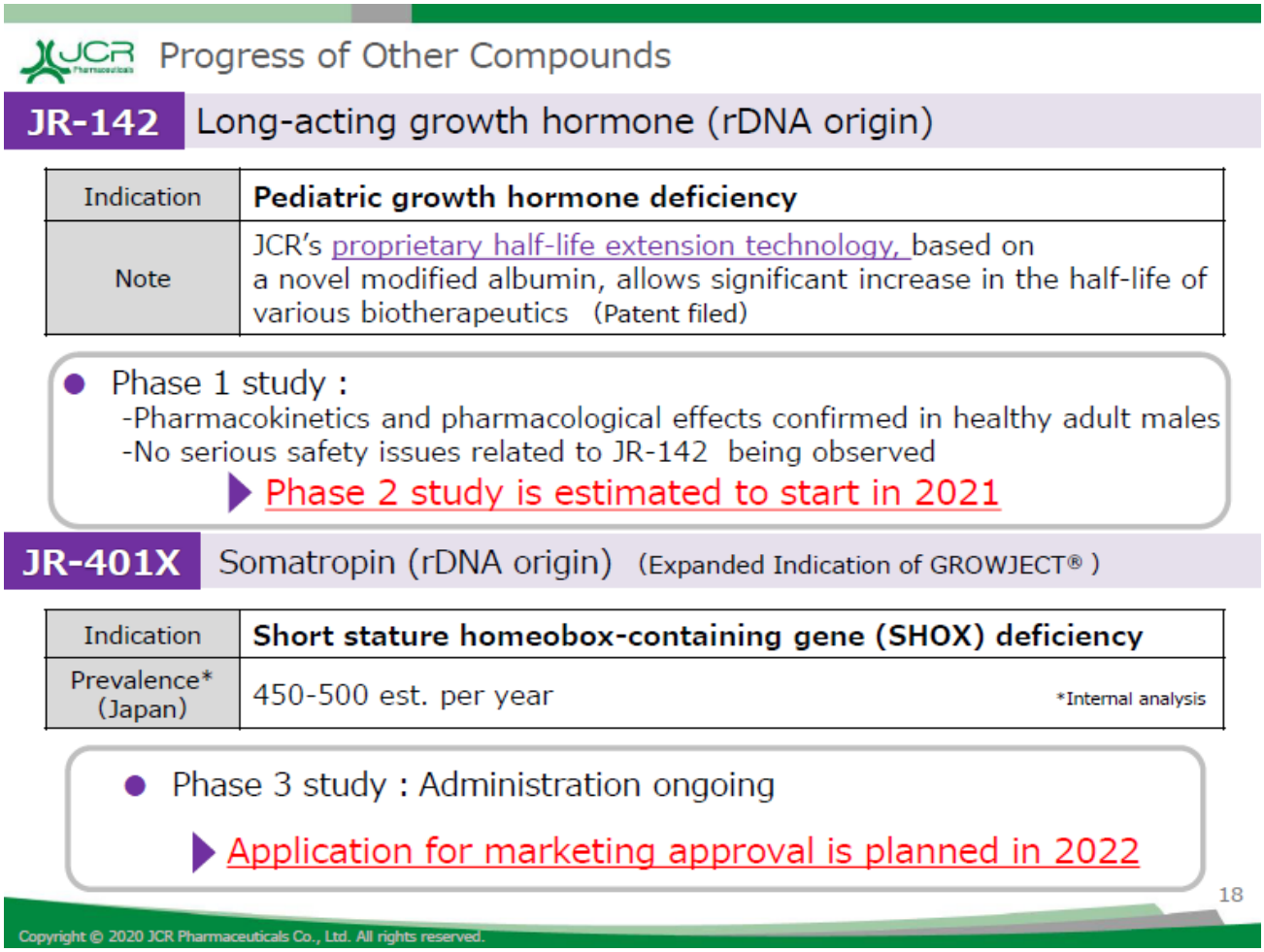
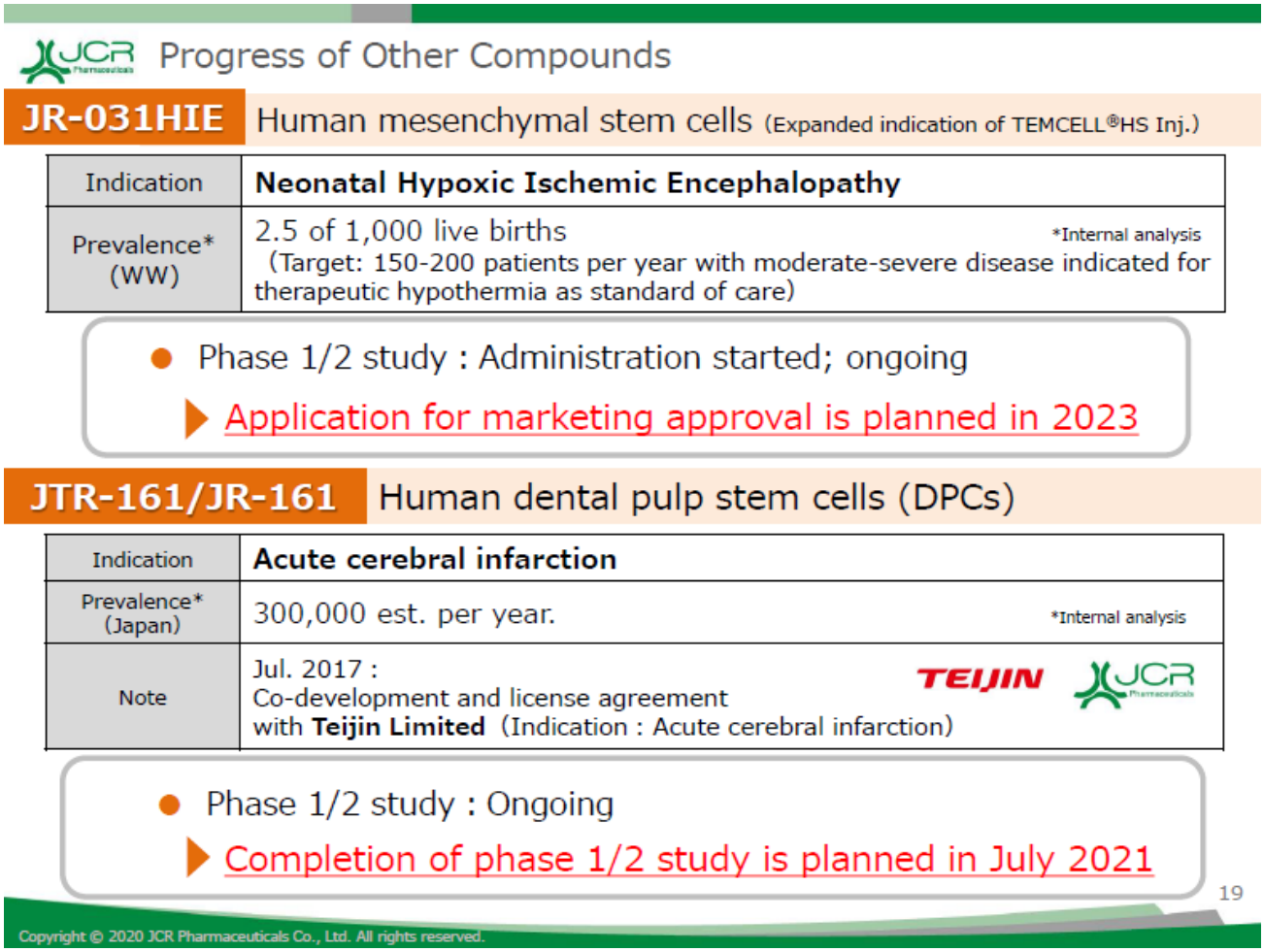
JR-441 is a treatment for type IIIA mucopolysaccharidosis. We do not currently have an enzymatic formulation to treat this disease type, so developing a therapeutic agent would be groundbreaking. In February of this year, new guidance for this disease was issued by the US FDA. With this guidance in mind, we are preparing to begin clinical trials as soon as possible.




This is a development product in the growth hormone field.

JR-142 capitalizes on our sustained circulation technology, as explained in the previous financial results briefing. The Phase I trial has been successfully completed, and the results have been as expected. Based on the results, we expect to be able to start a second-phase test in the next year with the aim of examining dosage and effectiveness.

SHOX deficiency is a hereditary disease characterized by short stature. We are currently recruiting patients for a study to research extending the indications of Growject to include this condition. If all goes according to plan, we expect to submit an application for manufacturing and marketing approval in 2022.

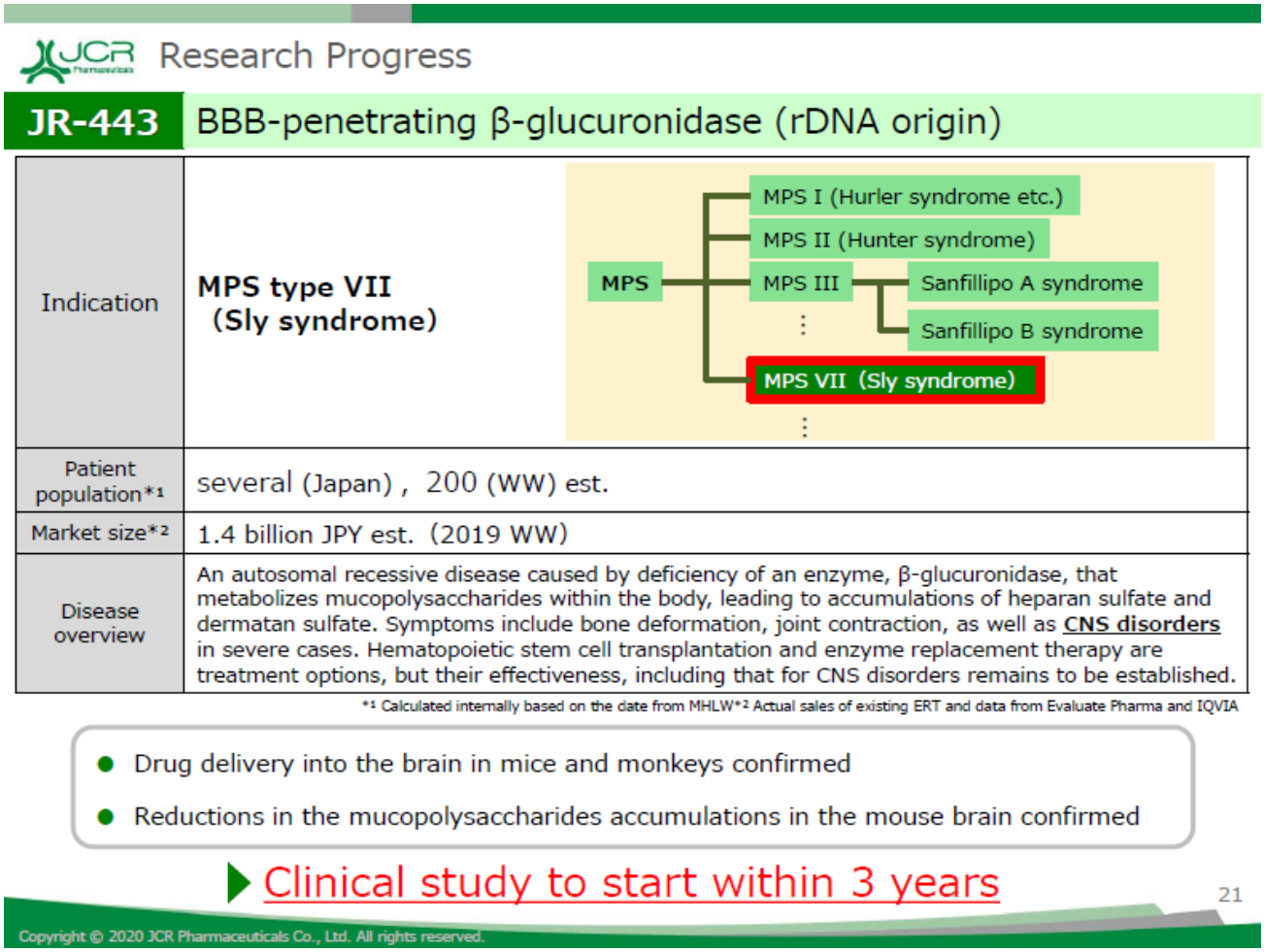



The last slide covers products for regenerative medicine and other products.

We are aiming to expand the indications for Temcell to include neonatal hypoxic ischemic encephalopathy. This is an extremely serious disease associated with newborns who experience asphyxia. We have begun clinical Phase 1/Phase 2 trials and we are observing the progress of this disease. We aim to submit an application for marketing approval in 2023.

JR-161 is being co-developed with Teijin Pharma, and Phase 1/Phase 2 trials are scheduled to be completed in July 2021.

This concludes my presentation.



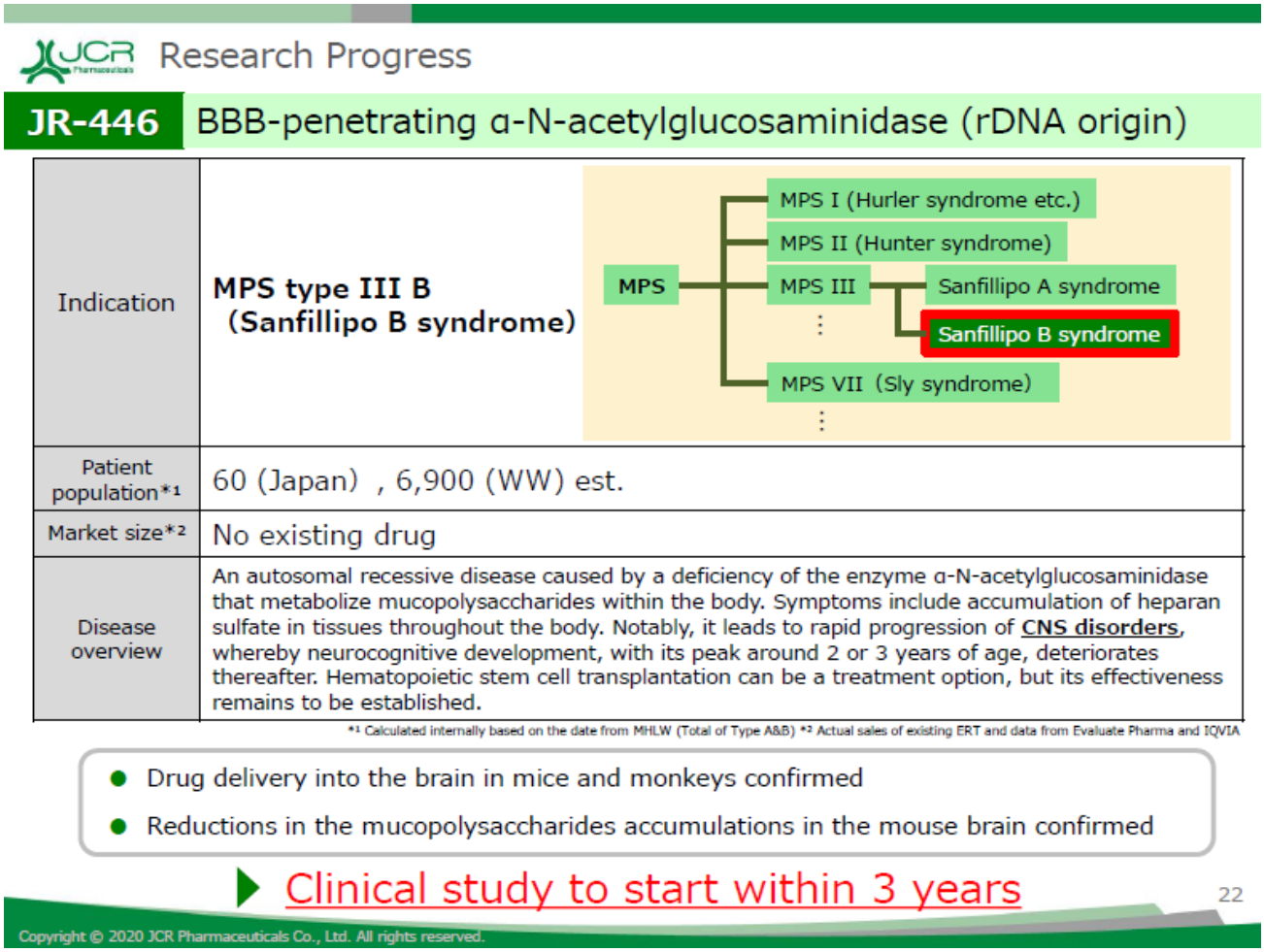

**Sonoda:** I will talk about the progress made at the research stage.

Progress has been made in several research areas. The first is JR-443, a recombinant β-glucuronidase that crosses the blood–brain barrier. This pipeline is targeting mucopolysaccharidosis type VII (Sly syndrome). JR-141 and JR-171 also target mucopolysaccharidoses, but this one targets type VII (Sly syndrome) in particular.

We are doing a lot of work in treating such diseases, and they are all rare diseases. Sly syndrome is classified as ultra-rare. Therefore, I think that of the treatments we are developing, this one will have the fewest patients. In addition, enzymes have already been launched in Europe and the United States for this disease.

However, the existing enzyme preparation does not pass through the blood–brain barrier, so it is ineffective for patients with severe disease who have CNS disorders. We intend for JR-443 to cross the blood–brain barrier.

Brain transferability of this drug has already been confirmed in mouse and monkey models. In addition, we have confirmed a decrease in the amount of mucopolysaccharide accumulated in the brain in the mouse disease model. Currently, the R&D laboratories are working on the development of manufacturing methods and nonclinical trials, with the aim of starting clinical trials within three years.




JR-446 is a genetically modified alpha-N-acetylglucosaminidase. This compound also utilizes blood–brain barrier transit techniques. The target disease is also mucopolysaccharidosis, this time type IIIB (Sanfilippo syndrome type B). There are no existing treatments, and given the number of patients we see here, there is definitely an unmet need for a treatment.

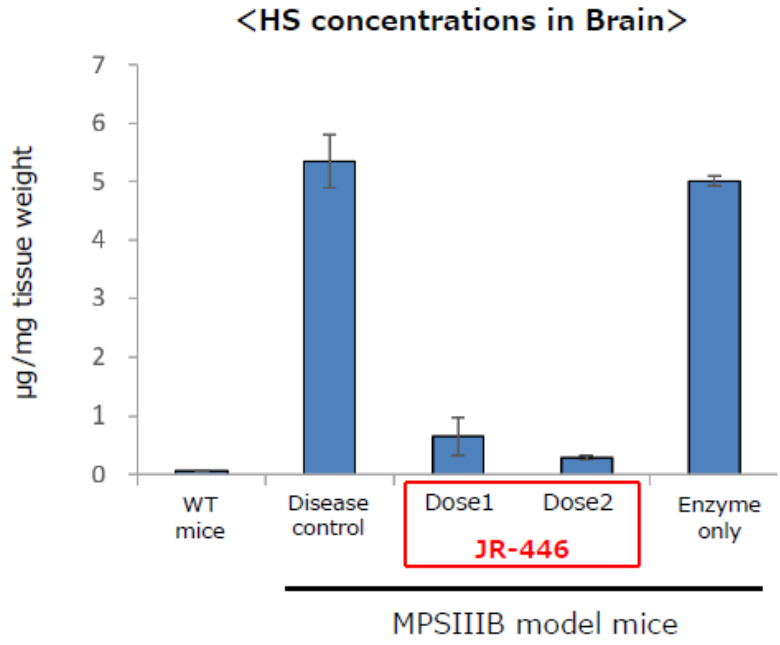
In addition, even among mucopolysaccharidoses, this condition is characterized by pronounced central neuropathy. Therefore, technology to cross the blood–brain barrier is essential in developing a treatment.

JR-446 has been developed by using techniques that have been proven to cross the blood–brain barrier in humans, as in JR-141. It is therefore likely that the results will be favorable for Sanfilippo's syndrome type B. Blood–brain barrier transfer was also confirmed in mouse and monkey models. In addition, we have confirmed a decrease in mucopolysaccharide accumulation in the brain in disease model mice. We are considering the possibility of starting clinical trials within three years.

Research Progress

**JR-446** BBB-penetrating α-N-acetylglucosaminidase (rDNA origin)

■ **The results of drug efficacy study in MPSIIIB model mice**

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For this disease, I would like to show some results from the mouse model. What is shown here is heparan sulfate levels in model mouse brains. Heparan sulfate accumulates in the brain in these diseases. The vertical bars indicate the amount of the heparan sulfate.

The left-hand side is from a normal brain, with hardly any accumulation. Next is a disease model mouse. On a rough scale, the figures show a range between five and six, which is the amount of heparan sulfuric acid that has accumulated up to this point.

We will move on to the far right-hand bar chart. This shows the result after treatment with enzymes that cannot cross the blood–brain barrier. As you can see, the amount of heparan sulfate in the brain remains unchanged.

Dose one and dose two of JR-446 represent different dosing types, but both were administered intravenously. As you can see, there is very marked decrease in heparan sulfate concentration.

On the other hand, there is an interesting thing about JR-446. This enzyme, called N-acetylglucosaminidase, is known to be extremely difficult to manufacture. In general, biopharmaceuticals and protein-based pharmaceuticals are made using CHO cells, but the common understanding is that with CHO cells, N-acetylglucosaminidase is very difficult to express and, as a product, it is difficult to make.

We have been considering various ways to crack this problem. As we haven't yet issued any patents, we cannot disclose detailed information. However, we have made a breakthrough, and we have succeeded in greatly increasing the amount of protein expressed. This will allow us to produce JR-446.

I believe that the combination of blood–blood barrier transit and other technologies has allowed us to move forward with development of JR-446.

## Research Progress

### JR-162 J-Brain Cargo®-applied acid α-glucosidase (rDNA origin)

| | |
|---|---|
| Indication | **Pompe disease** |
| Patient population*1 | 80 (Japan), 10,600 (WW) est. |
| Market size*2 | 3 billion JPY est. (2019 Japan), 110 billion JPY est. (2019 WW) |
| Disease overview | An autosomal recessive disease caused by a deficiency of the enzyme acid α-glucosidase that causes an **accumulation of Glycogen in muscle cells and nerve cells.** The infantile onset manifests as suckling and muscle force lowering in postnatal 2 months. Natural history suggests a life expectancy of less than 18 months due to cardiac dysfunction and respiratory failure. Delayed onset cases present muscle weakness that involves respiratory muscles. Symptoms are multiple and systemic, including **CNS disorders**. |

*1 Calculated internally based on the date from MHLW *2 Actual sales of existing ERT and data from Evaluate Pharma and IQVIA

- Summary of Non-clinical study results :
  (from the presentation at the 16th Annual WORLD*Symposium*™2020 )
  - JR-162 has potentials to exert therapeutic effects on muscle weakness and respiratory dysfunctions caused by both myogenic as well as neurogenic myopathies

Phase 1/2 study is planned in 2023

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The following is JR-162, a genetically modified acid alpha-glucoside using J-Brain Cargo. The indication is Pompe disease. Pompe disease is also a lysosomal disease.

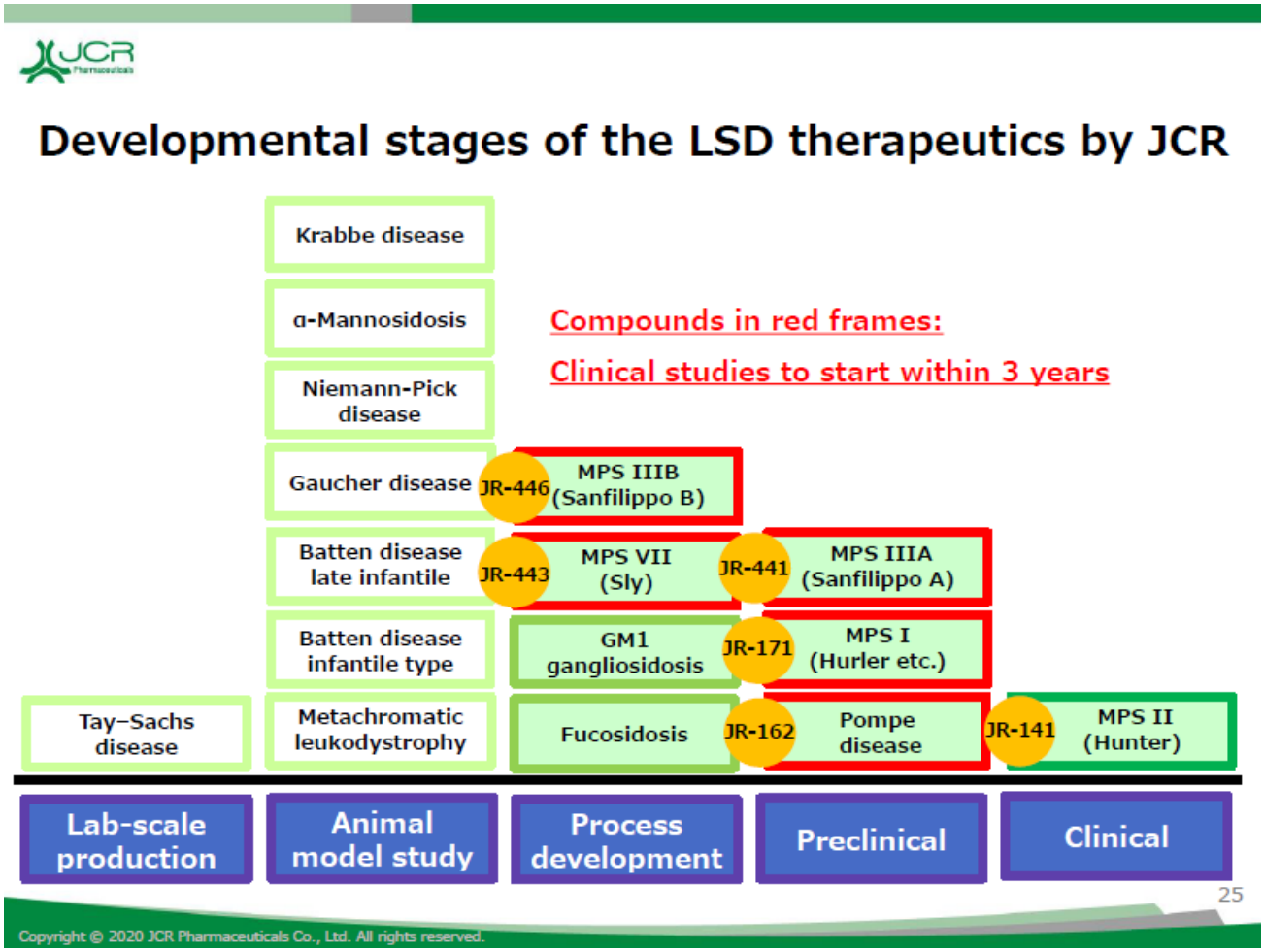
For Pompe disease, we have already launched first- and second-generation enzymes. JR-162 is our third-generation treatment.

Of these treatments, JR-162 has a unique characteristic. It is capable of delivering drugs not only to muscle tissues but also to nerve tissues and the brain.

In addition to damaging the muscles themselves, this disease also results in neurological impairment. Therefore, we believe that true efficacy can only be obtained when we are treating both.

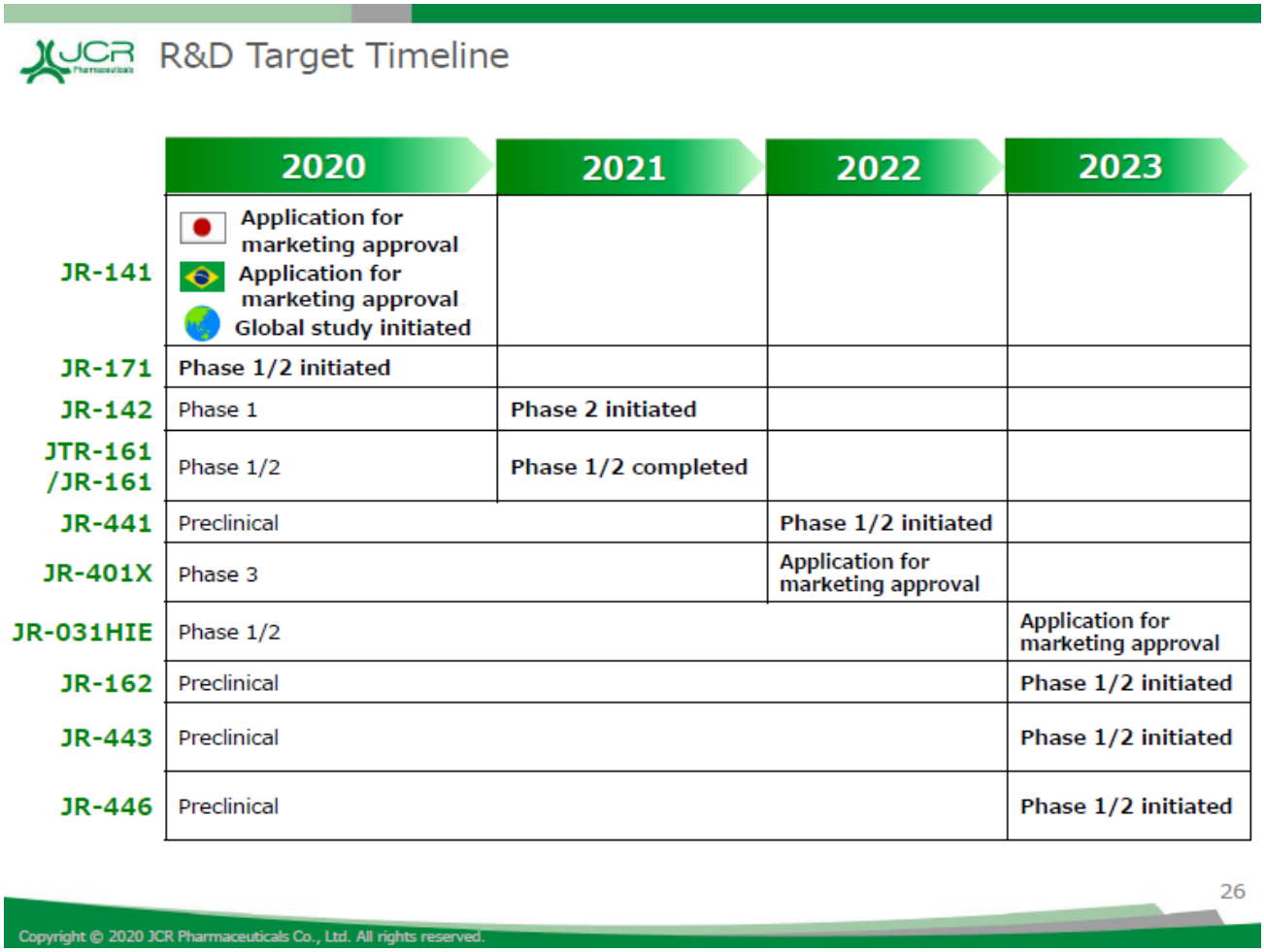
We believe that only JR-162 can do so. The strongest point of JR-162 for patients with Pompe disease is that it can be effective in both muscle- and nerve-related symptoms.

We are preparing to begin clinical trials of JR-162 in 2023.




This is a list of the lysosomal diseases that JCR is working on, by development stage. This time, JR-446 and JR-443 are in the middle column.

There are many other candidates for lysosomal disease, but all of these products are being researched and developed. We are making every effort to move forward as soon as possible, to get each one to the next stage.




This is the expected timeline for future research and development.

From 2020 to 2023, five new products are scheduled to enter clinical trials.


We would like to create as many new drugs in the rare-disease area as possible with these three platforms: genetically modified protein-based drugs, cell-based and regenerative medicines, and gene therapy.

Although we are not reporting any new developments in the field of genetic therapy today, our research and development in this area is proceeding very smoothly. In the near future, we are also working on preparations for a trial facility.

In the field of cell-based medicine, we are also promoting the development of stem cells derived from tooth pulp, the next step from Temcell. We have also started research and development with a view to new cell medicines.

Based on these three platforms, I would like to move forward with the realization of "health care that will help you live with orphan diseases." This is all.

**Toru Ashida:** I am Toru Ashida, Director in charge of Corporate Strategy. Let me now explain the three-year, medium-term management plan, "Revolution," beginning in FY2020.

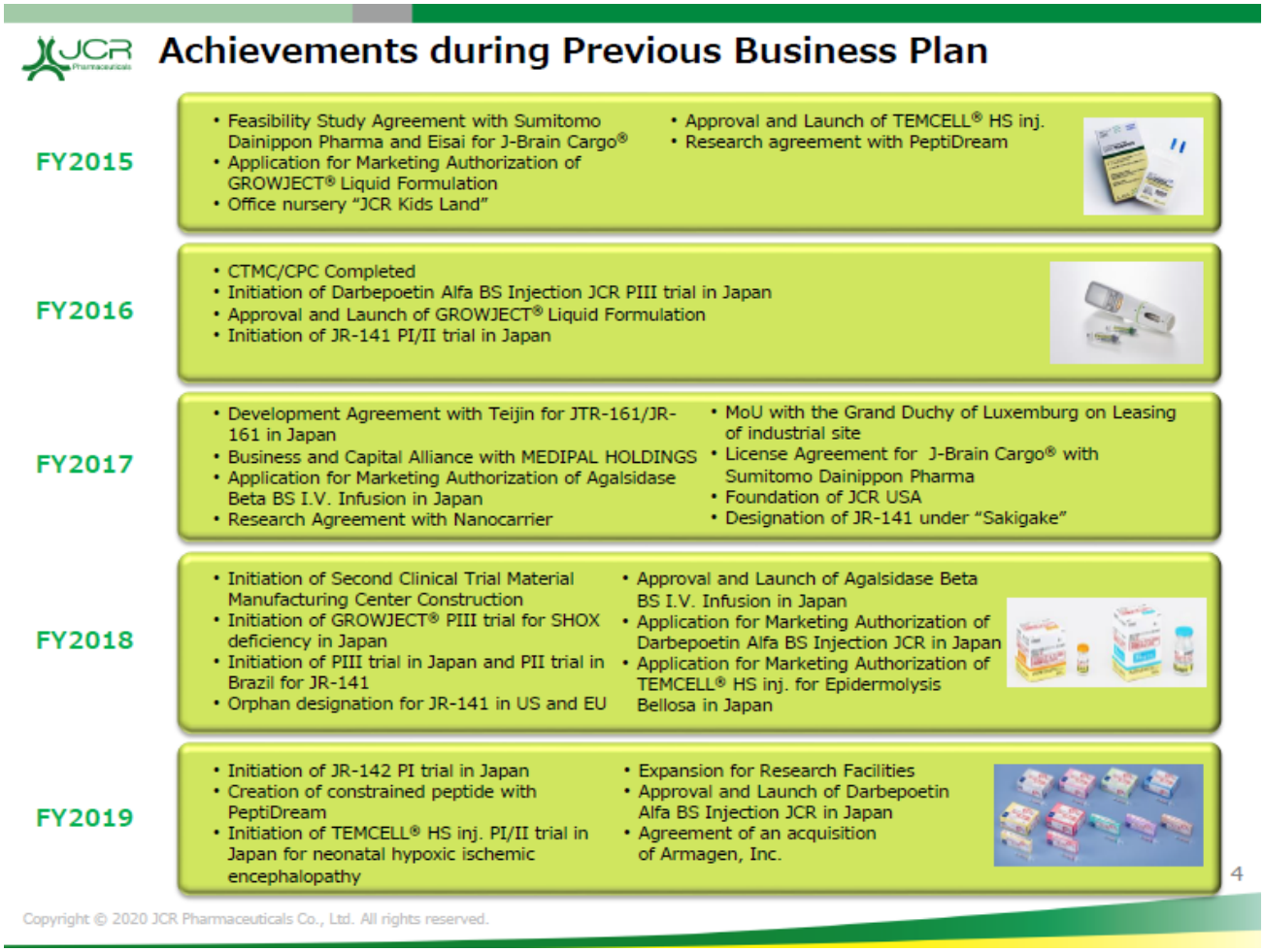
We achieved a great deal in the previous medium-term management plan, "Leap," from FY2015 to FY2019. Based on these achievements, we have set our sights on significant growth over the 10-year period beginning in FY2020.

In the preparation of the medium-term management plan, which I will present today, we reviewed the history of our founding, the conditions we have faced, the potential for future growth, and the issues that need to be resolved. Through dialogue with all divisions in the Company and multiple business meetings at the

management level, we have created a new medium-term management plan based on the keyword "Revolution."

The bands indicate the image of future growth, while green indicates corporate color, and amber indicates a bright future, knowledge, and well-being.

The composition of this slide is shown here. After summarizing the previous medium-term management plan, "Leap," I will explain how we are looking ahead to the next 10 years in the "Toward 2030" section. Finally, I would like to explain the three-year, medium-term management plan, "Revolution," beginning in FY2020.




First, I would like to give a summary of the previous medium-term management plan, "Leap."

As you can see, over the five years from FY2015, we made a number of achievements that will serve as the foundation for our future growth.

In FY2015, we acquired manufacturing and marketing approval for four products, including Temcell, and launched these products. In FY2016, we completed construction of a clinical trial drug manufacturing center and cell processing center. We have also made aggressive capital investments in production and research.

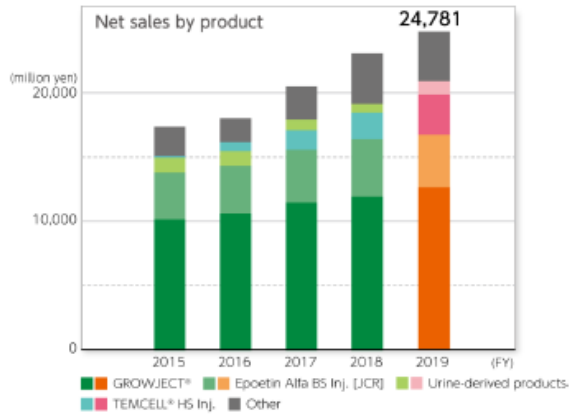
In addition, we have implemented a variety of initiatives, including the establishment of on-site daycare centers, so that each and every employee can work energetically and vigorously.

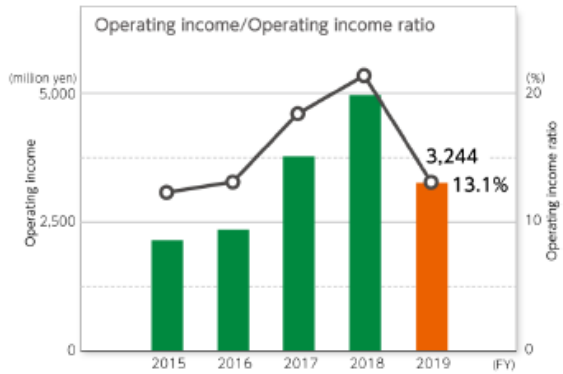
In R&D, we began clinical trials of JR-141 in FY2016 and actively engaged in researching orphan diseases.

## Achievements during Previous Business Plan

**FY2015 to FY2019**

| | | |
|---|---|---|
| Sales | : ¥17.4 bil | to ¥24.7 bil |
| Operating income | : ¥2.15 bil | to ¥3.24 bil |
| Operating income to sales | : 12.3% | to 13.1% |

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As a result of these efforts, in the five years of "Leap," we increased our net sales from JPY17.4 billion to JPY24.7 billion, and our operating income from JPY2.1 billion to JPY3.2 billion.

## Achievements during Previous Business Plan

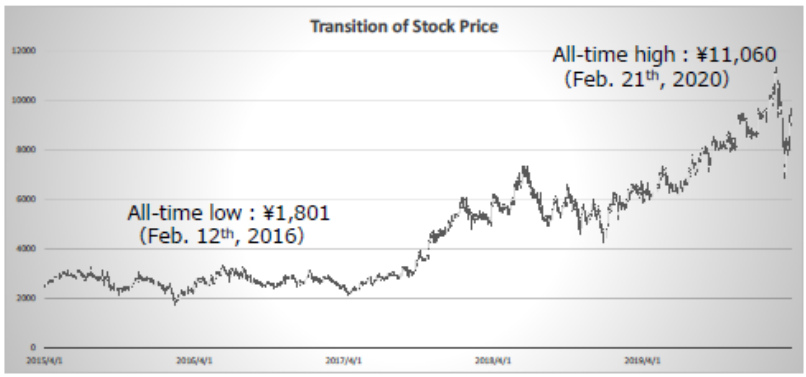
| FY | Num. Employees (Non-consolidated) | Avg. Age | Duration of Service | Avg. Annual Salary |
|---|---|---|---|---|
| 2019 | 654 | 41.4 | 8.9 | ¥8.3 mil |
| 2018 | 613 | 41.4 | 8.8 | ¥8.3 mil |
| 2017 | 540 | 41.6 | 9.0 | ¥8.2 mil |
| 2016 | 522 | 41.4 | 8.6 | ¥8.1 mil |
| 2015 | 487 | 41.1 | 8.3 | ¥8.1 mil |

**As of Mar. 31,2019**

No. of Employees : 654
Avg. age : 41.4
Avg. salary : ¥8.3 mil

**As of Mar. 31, 2016**

No. of Employees : 487
Avg. age : 41.1
Avg. salary : ¥8.1 mil




**As of Mar. 31, 2019**
Close : ¥9,420
Market Cap. : ¥305.4 bil

**As of Apr. 1, 2015**
Close : ¥2,480
Market Cap. : ¥80.4 bil

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The number of employees increased from 487 to 654, and the average annual income increased from JPY8.09 million to JPY8.3 million. In this way, we have been actively investing in human resources.

Stock prices, on the back of our growth expectations, rose from JPY2,480 at the beginning of FY2015 to JPY9,420 at the end of FY2019. On February 21, 2020, we reached a record high of JPY11,060.

## General Overview of Previous Business Plan

### JCR achieved substantial results for the next stage during “HIYAKU” with “Speed First” spirit.

- Approval and launch of TEMCELL® HS inj. ,GROWJECT® liquid formulation, Agalsidase beta BS I.V. infusion and Darbepoetin alfa BS injection
- On-track progress of JR-141 PI/II trial in Japan and PII trial in Brazil
- PII trial in Brazil, the first global development for JCR, successfully initiated with the support of the patient advocacy group and medical experts
- Acquisition 100% ownership of ArmaGen Inc. to fortify the lysosomal enzyme replacement therapy (ERT) franchise
- Construction of CTMC (Clinical Trial Material Center) and its annex, CTMC2, for lysosomal drug candidates
- Construction of CPC (Cell Processing Center) for DPC (Dental Pulp Cell) regenerative medicine candidate

### To strengthen licensing activities for rare disease business to maximize corporate value





This slide summarizes our progress and presents our challenges for the future.

As I have explained, we have achieved many results during the period of "Leap." Among these, we believe that our ability to quickly begin clinical trials in Brazil, particularly with a relative lack of experience in global development, is a noteworthy achievement.

The acquisition of ArmaGen, Inc. in the US also had significant implications for our development of treatments for lysosomal diseases, and this acquisition completely resolved all potential intellectual property risks.

Based on these achievements, we will strengthen our licensing activities to maximize business value, which is an issue to be addressed in the future.

## Sustainability

**Through our business activities centered on the field of pediatric rare diseases, we will strive to "contribute to unmet medical needs" and enhance activities related to "Rare Diseases: RD, ESG".**

Initiatives

### Rare Diseases (RD)
Conduct awareness-raising activities as a company promoting research and development of orphan drugs.

RAREDISEASEDAY.ORG

Launched the "RARE DISEASE" project, with "What JCR can do" as its motto, promote awareness-raising activities and initiatives for Rare Disease Day.

### Society (S)
Maintain a comfortable working environment and conduct initiatives aimed at society.

もみじの家

To contribute to addressing the unmet medical needs of patients all over the world, we support the GLOBAL FOUNDATION FOR LIFE SCIENCE (a non-profit foundation in Switzerland), the Mother and Child Health Encouragement Award, and Momiji House (a short-term residential facility for severely ill children and their families).

### Environment (E)
Implemented efforts to reduce the environmental impact of business activities.

### Corporate Governance (G)
Establish and operate a system to maintain legality, transparency and objectivity of management.

Contribution through our business | Promote the **"Realizing medical care for those living with rare diseases"**

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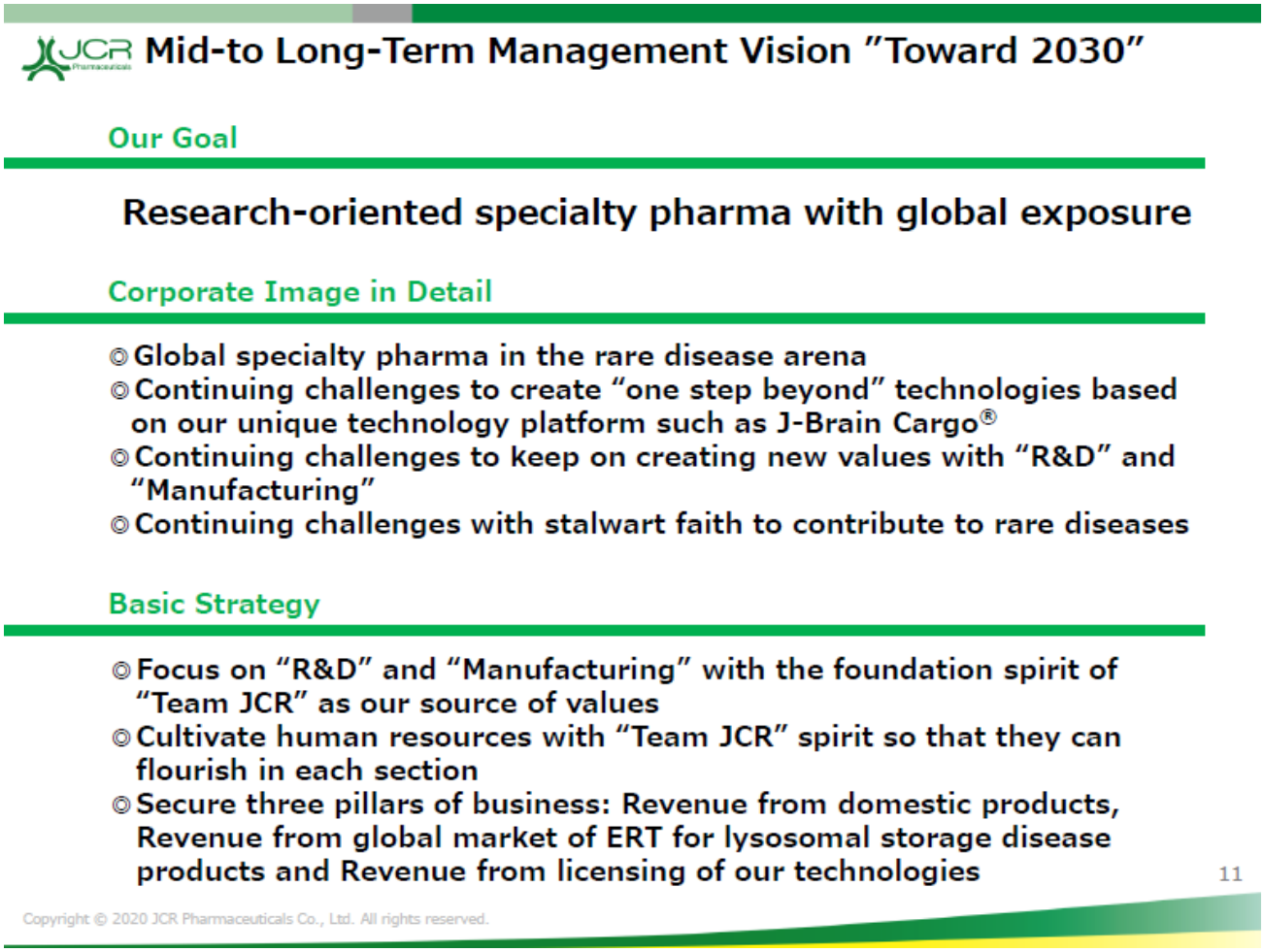
Next, I will discuss sustainability.

Recognizing that it is our duty to realize "health care that will help you live with orphan diseases," we have been engaged in a variety of initiatives, including research and development of orphan drugs and support for insured health care.

In addition, based on the belief that each and every employee is a source of value, we have worked hard to create a supportive work environment. In addition, we have been working on environmental initiatives and strengthening corporate governance.


Let me now explain how we look at the next 10 years.


Our corporate philosophy is to "contribute to people's health through pharmaceuticals," and this philosophy is our raison d'être. We recognize that it is our duty to combine our unique R&D and manufacturing capabilities to take on the challenge of orphan diseases for patients and children around the world.

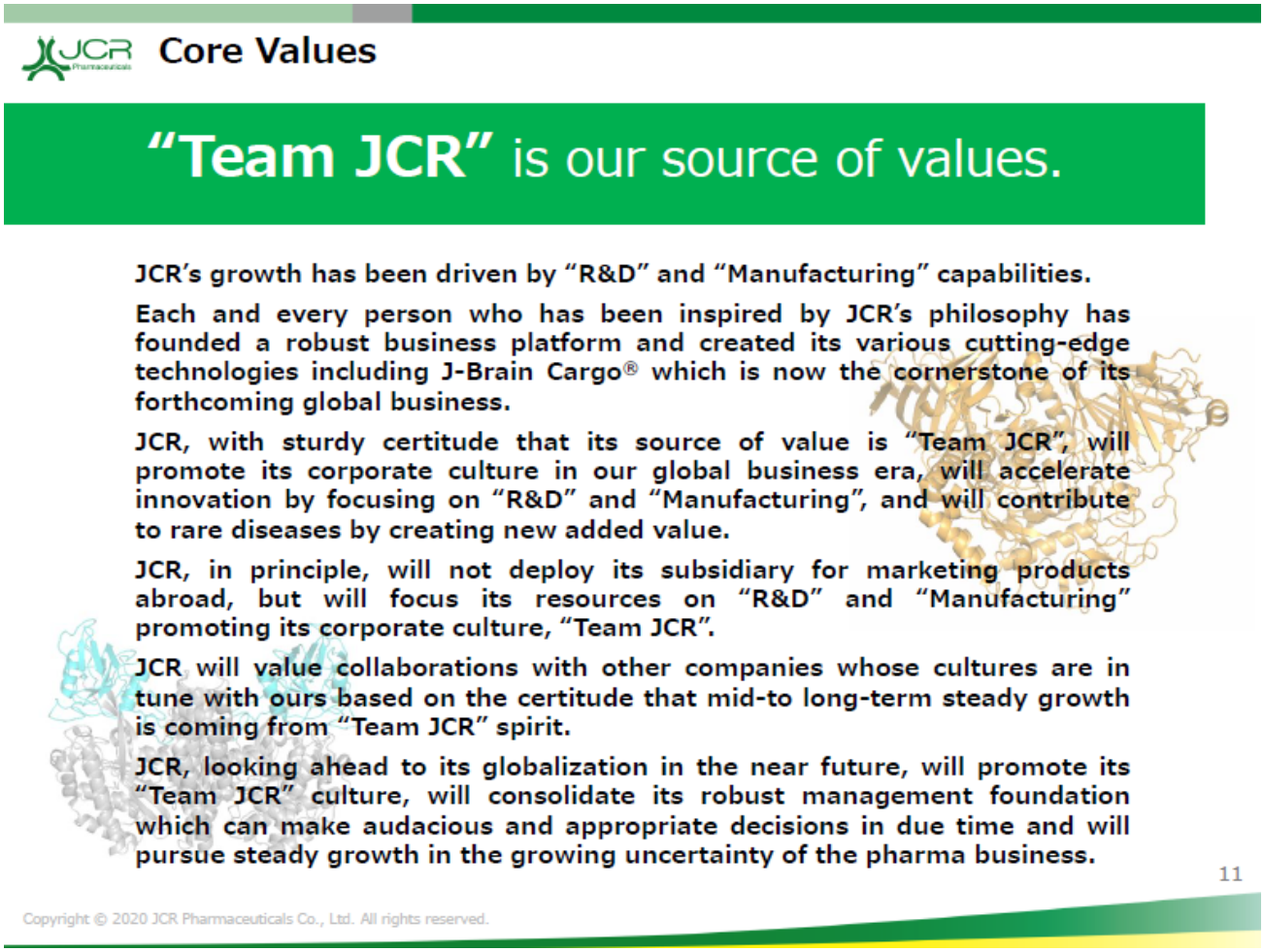



Our vision for the future is to continue to be an R&D-oriented Company with a strong global presence. The corporate image to be realized is to be a global specialty pharmaceutical Company in the orphan disease field, and to continue to push forward with the creation of technologies that constantly go one step ahead of other companies. We want to continue in the trend of J-Brain Cargo, and other proprietary core technologies. We believe that we must continue to challenge ourselves, without compromise, to create new value through R&D and manufacturing. This is based on a strong determination to contribute to the orphan disease field.

As a basic strategy to achieve this, we will focus on research and development and manufacturing with a small number of talented people. Team JCR, which shares and develops our corporate culture since our founding, is the source of our value.

We will also cultivate human resources so that each team JCR can shine.

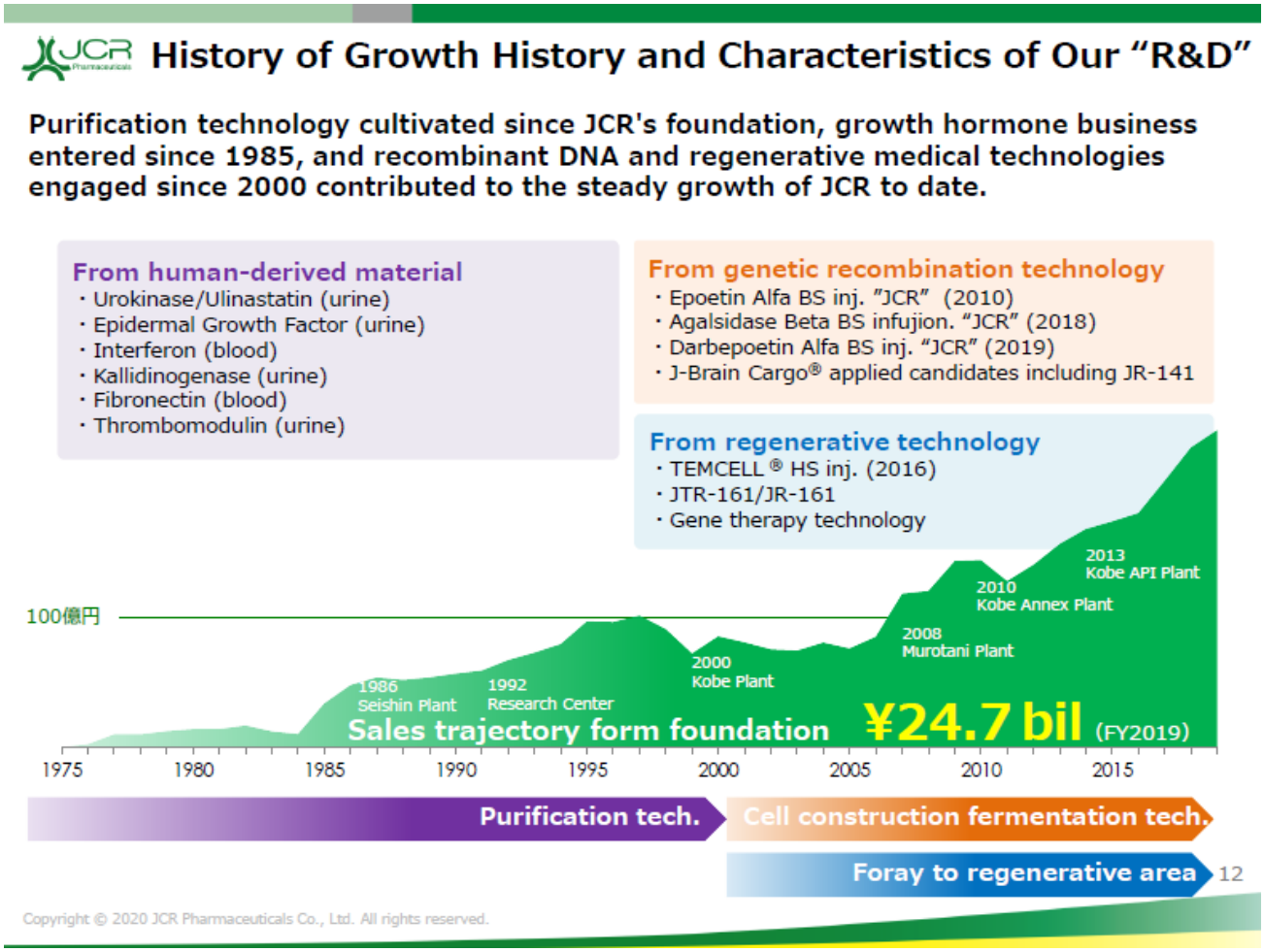
We will secure three major revenue streams: domestic products, such as growth hormones; global revenues from the treatment of lysosomal disease; and licensing revenues from the out-licensing of core technologies.




In achieving this goal, we have thought about what our core values are. The source of our value is team JCR. Each of our employees, who share the corporate culture that we have grown through manufacturing and R&D, has helped establish a stable management foundation, created a variety of proprietary technologies, and served as the foundation for our growth worldwide. Based on our conviction that the source of value is JCR, we will further develop our corporate culture in the global era and contribute to the area of orphan diseases by creating new value.

As a general rule, we will not develop our own overseas sales bases for the development of team JCR's corporate culture. In collaboration with other companies, we will focus on whether or not our corporate culture is consistent in order to achieve stable growth over the medium to long term.

In anticipation of full-fledged globalization in the near future, we will strive to develop Team JCR's corporate culture, establish a solid management foundation, and aim for sustainable and stable growth in the increasingly uncertain pharmaceutical industry.




This section briefly reviews our progress.

In the first 25 years since our founding in 1975, we have developed technological know-how based on research and development into urinalysis and hematology.

Against the backdrop of concerns over bio-based substances since the 1990s, we have shifted the focus of our research and development activities to biotechnology and regenerative medicine since the early 2000s. As a result, we successfully launched treatments such as epoetin alfa and regenerative therapies such as Temcell.

## JCR's "R&D"

- Steady R&D activity with robust business base from growth hormone revenue (since 1993) .
- Purification technology as our fundamental technology starting from urokinase business, cultivated through over 20 years of R&D using human-derived materials such as urine and blood.
- Recombination DNA technology since the early 2000s when JCR shifted to the area of its R&D focus. Our first product was the first domestic biosimilar in Japan, Epoetin Alfa BS launched in 2010.
- JCR has been engaging in the development of drugs for lysosomal storage diseases from early 2000s.
- Mesenchymal stem cell research since 2003. Our first product was the first allogenic cell product in the world, TEMCELL® HS, launched in 2016.
  JCR has been engaging in the development of dental pulp cells for acute cerebral infarction.
- Cutting-edge technologies J-Brain Cargo®, Modified albumin-based long-acting technology, J-Mig (High Expression system), and J-Glyco S/M (Glycosylation technology) resulting from thorough and repeated hypothesis verification.



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At the same time, we reviewed our R&D activities and asked what was distinctive. First, we were able to conduct R&D activities based on a stable business foundation in the growth-hormone business.

Another distinguishing characteristic of our R&D activities is our refined technology. We have cultivated this through our research and development of bio-derived materials, our genetically modified technologies accumulated over the past 20 years, our research in rare diseases, and our efforts in regenerative medicine.

The most important point is our attitude toward science. We repeatedly verify our hypotheses. This has enabled us to create J-Brain Cargo and other fundamental technologies.

## JCR's Potential -J-Brain Cargo®-

**Effective drugs are desired for lysosomal storage diseases, a group of ultra-rare diseases.**

**With the development of our drug candidates utilizing JCR's proprietary Blood Brain Barrier (BBB)-penetrating technology, JCR expects potential expansion of market penetration.**

Estimated number of patients and size of market for five lysosomal storage diseases

| Indication | Number of patients※1 | | Market size※2 | |
|---|---|---|---|---|
| | Japan | World | Japan (2019) | World (2019) |
| MPS II (Hunter) | 250 | 7,800 | ¥7.6 bil | ¥87.0 bil |
| Pompe | 80 | 10,600 | ¥3.0 bil | ¥110.0 bil |
| MPS I (Hurler/Hurler Scheie/Scheie) | 60 | 3,600 | ¥1.6 bil | ¥28.0 bil |
| MPS IIIA (Sanfilippo A) | 60 | 6,890 | — | — |
| MPS IIIB (Sanfilippo B) | | | | |

※1 Calculated internally based on the date from MHLW ※2 Actual sales of existing ERT and data from Evaluate Pharma and IQVIA

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Regarding the potential of our Company's core technologies, here is an example of a drug for lysosomal disease that uses J-Brain Cargo.

The market for orphan diseases is generally considered to be small, due to the small number of patients. Lysosomal disease, in particular, is a group of disorders with a very small number of patients, but for which there are either no drugs, or no drugs effective in treating central nervous system symptoms. These are important clinical challenges. Effective drugs are needed.

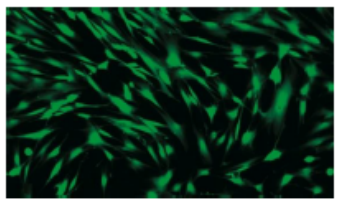
Our proprietary blood–brain barrier transit technology can provide an effective therapeutic agent in the field of lysosomal disease. This will not only greatly assist those affected by these diseases, but also provide us with tremendous growth potential.

In this way, the creation of core technologies will bring about significant growth. We will continue to focus on the development of original core technologies and explore new directions of R&D.

## R&D Direction

- Our current antibody-based J-Brain Cargo® technology is more amenable to target proteins rather than small molecules and nucleic acids.
- JCR is now investigating new modalities to enable those moieties to penetrate BBB by expanding the technological possibilities of J-Brain Cargo®, a transferrin receptor mediated drug delivery system.
  JCR seeks to create next generation of J-Brain Cargo® technologies through combination with other in-house technologies as well as through joint research with partners for constrained peptides and nanomicelles.
- JCR is also now investigating the possibility of gene therapy technology.
  Our R&D activities may lead to creation of new treatment options to address, for example, central nervous system diseases caused by a lack of essential molecules or genetic mutations.
- In the area of regenerative medicine, JCR is taking on the challenges in creating new treatment options for unmet medical needs leveraging our unique stem cell and recombinant DNA technologies.





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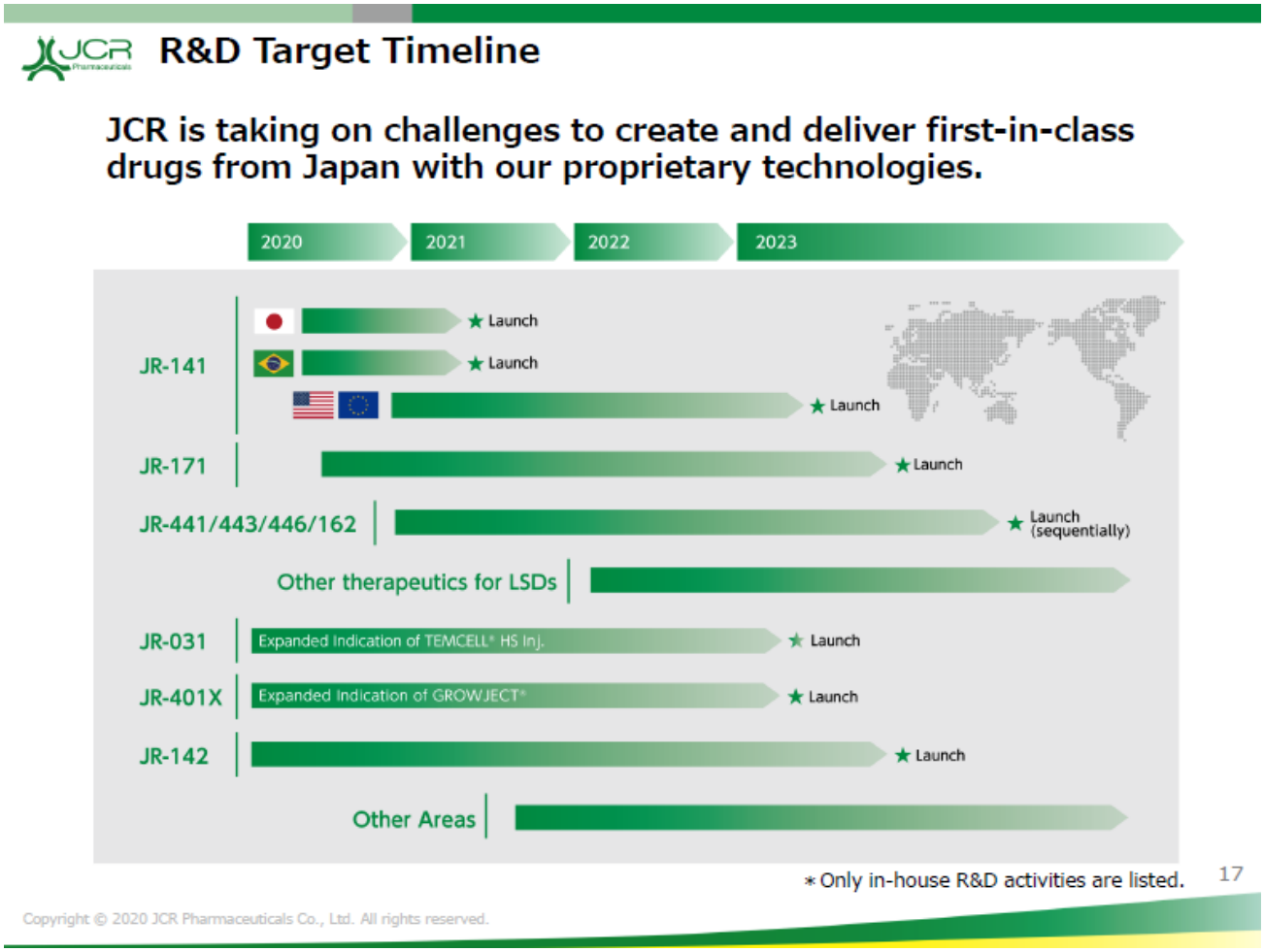
The direction of our future research and development is outlined below.

Since the present J-Brain Cargo is antibody-based, it is easy to combine it with a target protein. However, it needs to be reconsidered for small molecules and nucleic acids.

We are continuing our efforts to develop the technological potential of J-Brain Cargo, which is a drug-delivery mechanism that allows drugs to pass through the blood–brain barrier via the transferrin inhibitor. In addition to joint research with other companies in areas such as special cyclic peptides and nanomicelles, we are exploring a number of possibilities in-house that could become the next-generation J-Brain Cargo.

In addition to such research, we are also undertaking research and development in the field of genetic therapy. We hope such efforts will lead to the development of therapeutic agents for diseases caused by the lack of necessary substances in CNS tissue, and for other diseases caused by genetic mutations.

In regenerative medicine, we will apply the stem-cell and genetic-modification technologies we have cultivated to develop new treatment methods in therapeutic areas where conventional therapeutic approaches are not possible.




The timeline for medium- to long-term R&D is shown here.

We expect to enter a full-fledged global development stage over the next 10 years, and particularly from 2023. Our current focus on JR-141 and other lysosomal disease therapies will serve as a base for further expansion. We expect to launch a series of drug candidates for lysosomal disease on a global scale in the 2020s. In addition, we will focus on adding indications for JR-031 and developing products in the field of growth hormones.

We are also conducting basic research into other diseases. We will present further details in a timely manner.

Through these R&D activities, we expect to achieve full-fledged globalization from the latter half of the 2020s.

# Midterm Business Plan for 2020-2022
# “REVOLUTION”


From this slide, I will explain the three-year, mid-term management plan, "Revolution," which starts from this fiscal year.

This image reflects the strong commitment of each and every employee to contribute to orphan diseases, such as various types of lysosomal diseases, and the strong commitment that each and every employee must change in order to realize this commitment.


First, I would like to explain the keyword, "Revolution."

In 2025, we will celebrate the 50th anniversary of our founding. At the same time, we expect to achieve full-fledged globalization from the latter half of the 2020s. In order to achieve this, it is essential that each and every employee change in all aspects of our business activities, regardless of past successes. In light of this, we have set our keyword as "Revolution."

Looking toward the future and focusing on the revolution that is necessary, we have made "REVOLUTION into the Future" the phrase that sums up our vision.


First of all, I would like to point out the key management issues in the area of revolution.

We believe that providing a stable supply of high-quality pharmaceuticals is our greatest responsibility as a pharmaceutical Company, particularly in the area of orphan diseases.

In light of the increasing importance of orphan diseases, we have positioned the quality assurance system as a top management priority and will expand its quality and scope.

Looking ahead to the rapid expansion of our operations in the latter half of the 2020s, we will accelerate our efforts to address the following five, key management issues.

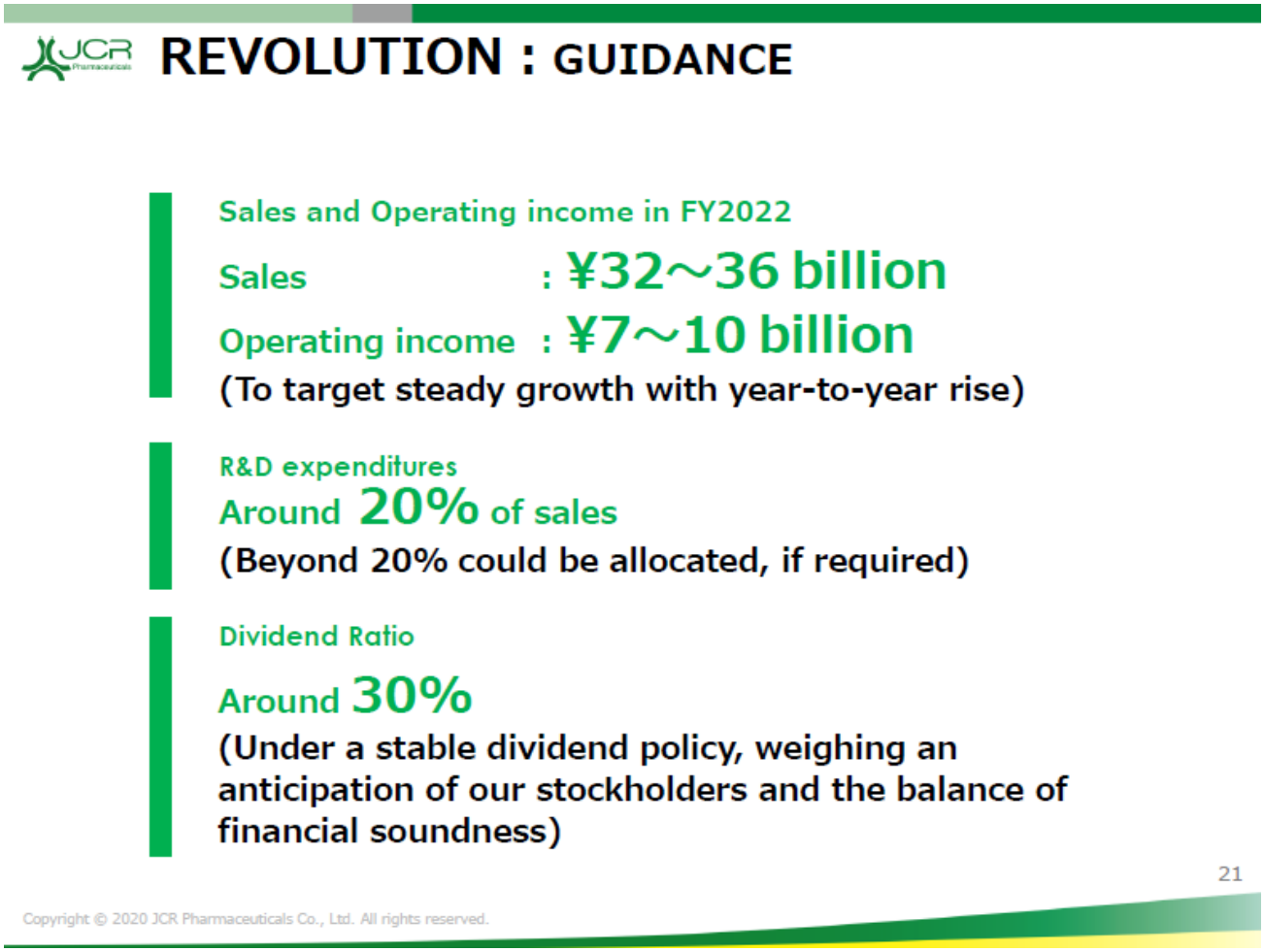
First, we recognize the importance of sustained growth of existing products until at least the time when lysosomal disease products will make a full-fledged contribution.

Second, based on the fact that it takes over 10 years to develop a drug, we will work to expand basic research with a view to the next stage of treatment of lysosomal disease.

Third, we will aggressively invest in R&D and capital expenditures for future growth, taking into account the business environment.

Fourth, in the area of orphan diseases, perhaps more than any other, health care professionals need medium- to long-term evidence relating to treatments. We will work on formulating product strategies, including building up evidence, to give health care professionals the information they require.

Finally, timely management decisions and face-to-face management are important for Team JCR, and we will continue to implement operational and organizational structural reforms, as well as human resource development, to enable management at an appropriate scale in the future business expansion phase.




Next, I would like to give more information about the area of innovation.

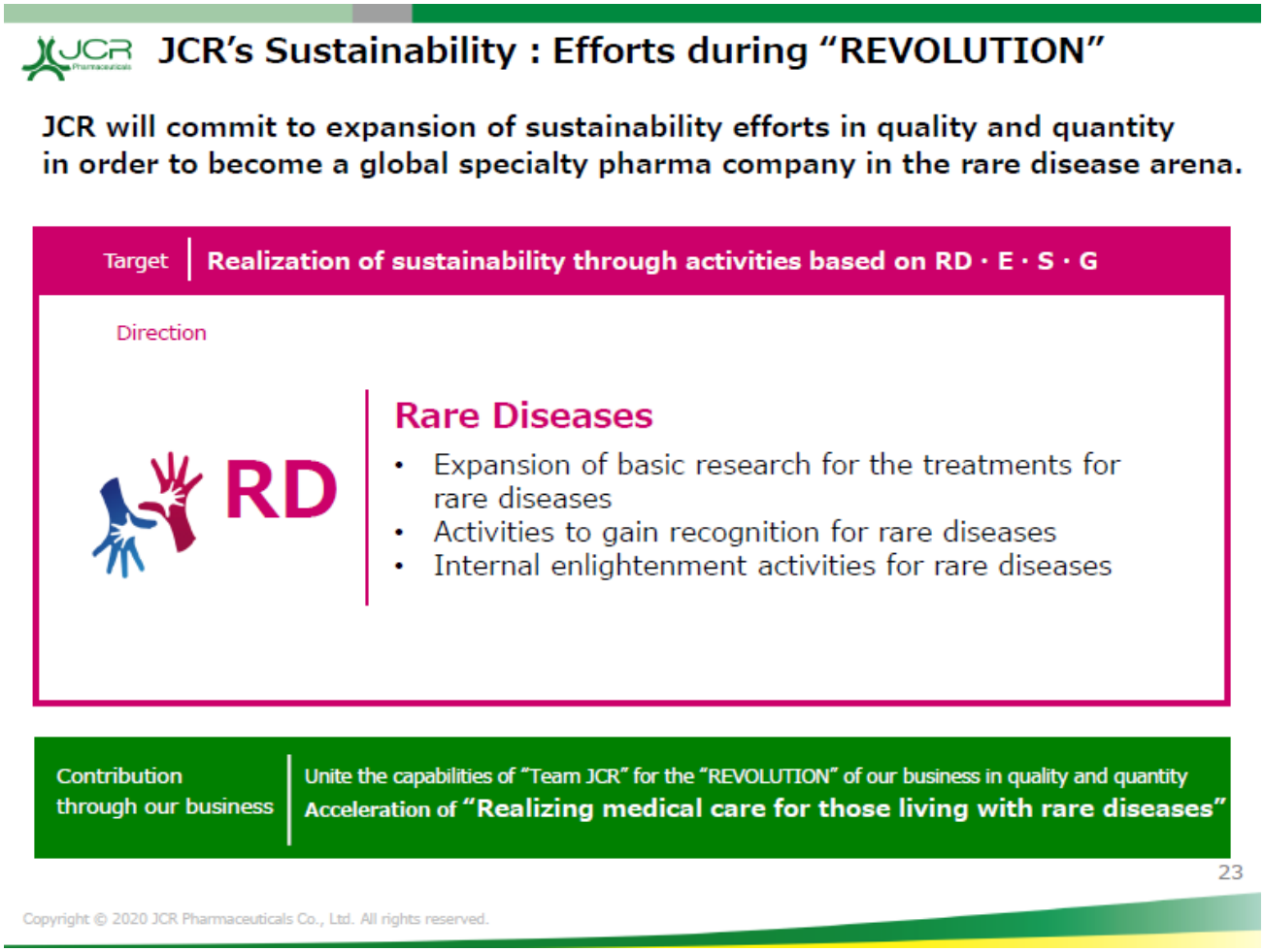
Our only KPIs will be sales, operating income, R&D expenses, and the dividend payout ratio, as we expect further growth in the future. With regard to net sales and operating income, we aim to achieve net sales of JPY32 billion to JPY36 billion, and operating income of JPY7 billion to JPY10 billion in FY2022, as well as stable, YoY growth.

Research and development expenses should be 20% of net sales. However, we allow this figure to be higher if necessary.

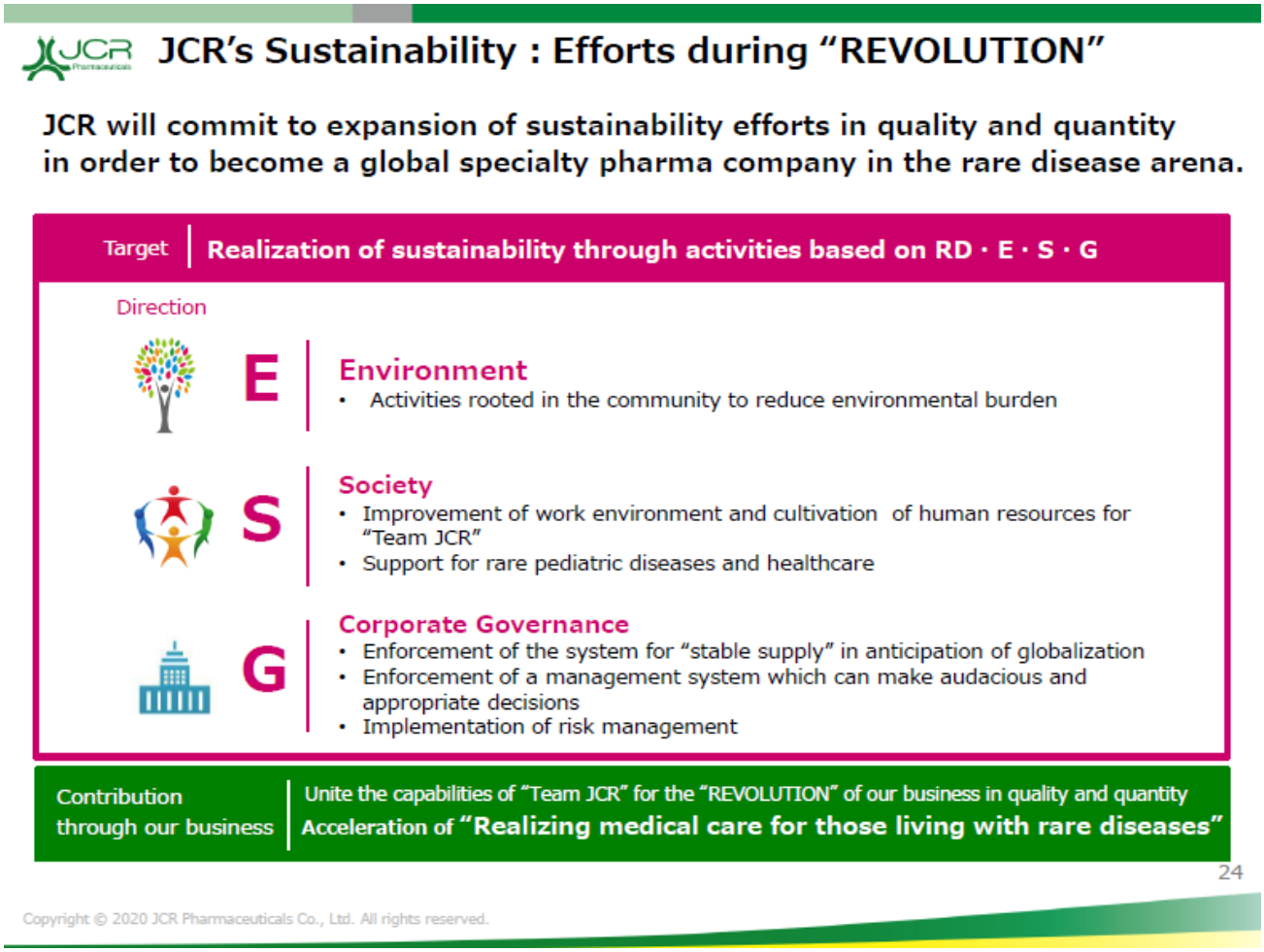
Finally, with regard to the dividend payout ratio, the Company's basic policy is to pay a stable dividend. There is an emphasis on the balance between shareholder returns that meet expectations and financial soundness, and as such the Company is targeting a dividend payout ratio of 30%.


Finally, I would like to explain our future sustainability initiatives.

Based on the recognition that "the realization of health care that will help you live with orphan diseases" is the foundation of value creation, we aim to achieve sustainability in line with the spirit of the sustainable development goal of "No one will be left behind." By focusing on issues related to readiness, the environment, society, and corporate governance in our business activities, and sharing the results with a wide range of stakeholders both inside and outside the Company, we aim to work toward realizing greater sustainability.




As a new initiative in the area of rare diseases, we will accelerate the development of drugs for lysosomal diseases and expand basic research that will lead to the development of other orphan-disease treatments.




As new initiatives in the fields of the environment, society, and corporate governance, we will promote activities to reduce environmental impact that are rooted in local communities. We will create a workplace environment where teams and employees can shine. We will continue to promote human resource development, strengthen the stable supply system of products for global expansion, and establish a solid management foundation that can make bold and appropriate decisions swiftly.

Through the above, we intend to accelerate the realization of health care that can live in harmony with orphan diseases, through qualitative and quantitative reforms of business activities that draw on the combined strengths of team JCR.

Thank you for your attention.

[END]

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***Document Notes***

1. *Portions of the document where the audio is unclear are marked as follows: [Inaudible].*
2. *This document has been translated by SCRIPTS Asia.*

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