JCR Pharmaceuticals Co., Ltd.

Q2 Financial Results Briefing for the Fiscal Year Ending March 2021 Presentation

November 2, 2020

Event Summary

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[Number of Speakers]	3

Shin Ashida	Representative Director Chairman, President and CEO
Akihiro Haguchi	Sr. Corporate Officer, Head of Administration Division, Executive Director, Administration Division
Kazunori Tanizawa	Corporate Officer, Executive Director, Development Division

Presentation

Ashida: I am Shin Ashida of JCR Pharmaceuticals Co., Ltd. Thank you very much for your interest in today's Financial Results Briefing for the Second Quarter of the Fiscal Year Ending March 2021.

At today's briefing session, each person in charge will provide an overview of The Results for the First Half of the Period Ending March 2021 and explain the status of R&D. I would like to discuss a license agreement for a drug for lysosomal disease.

At the financial results briefing held in May of this year, I stated that we expect to be able to sign a contract during the first half of the fiscal year. However, with the difficulty of negotiating face-to-face due to the COVID-19 pandemic, negotiations have not progressed as much as we had originally expected.

In addition, as I will expand upon later, this also affected our discussions with AstraZeneca regarding the domestic production of COVID-19 vaccine Bulk. Unfortunately, as a result, we were unable to conclude a contract by the end of September.

This has led to us revising our earnings forecasts. I would like to deeply apologize for any inconvenience to investors.

In R&D, JR-141, JR-171 and other new drug developments are progressing on schedule. We applied for marketing authorization for JR-141 in Japan at the end of September. The process in Brazil is also making steady progress toward filing for marketing approval this year. In addition, we are approaching the start of Phase 3 clinical trial globally. Regarding JR-171, a global clinical Phase 1/2 trial was accepted in July and a clinical trial was also accepted in Brazil in October. Following this, we will proceed with clinical trials in the US and elsewhere.

Since the development of these new drugs is proceeding very smoothly, we expect to conclude a license agreement for drugs for the treatment of lysosomal disease during the current term.

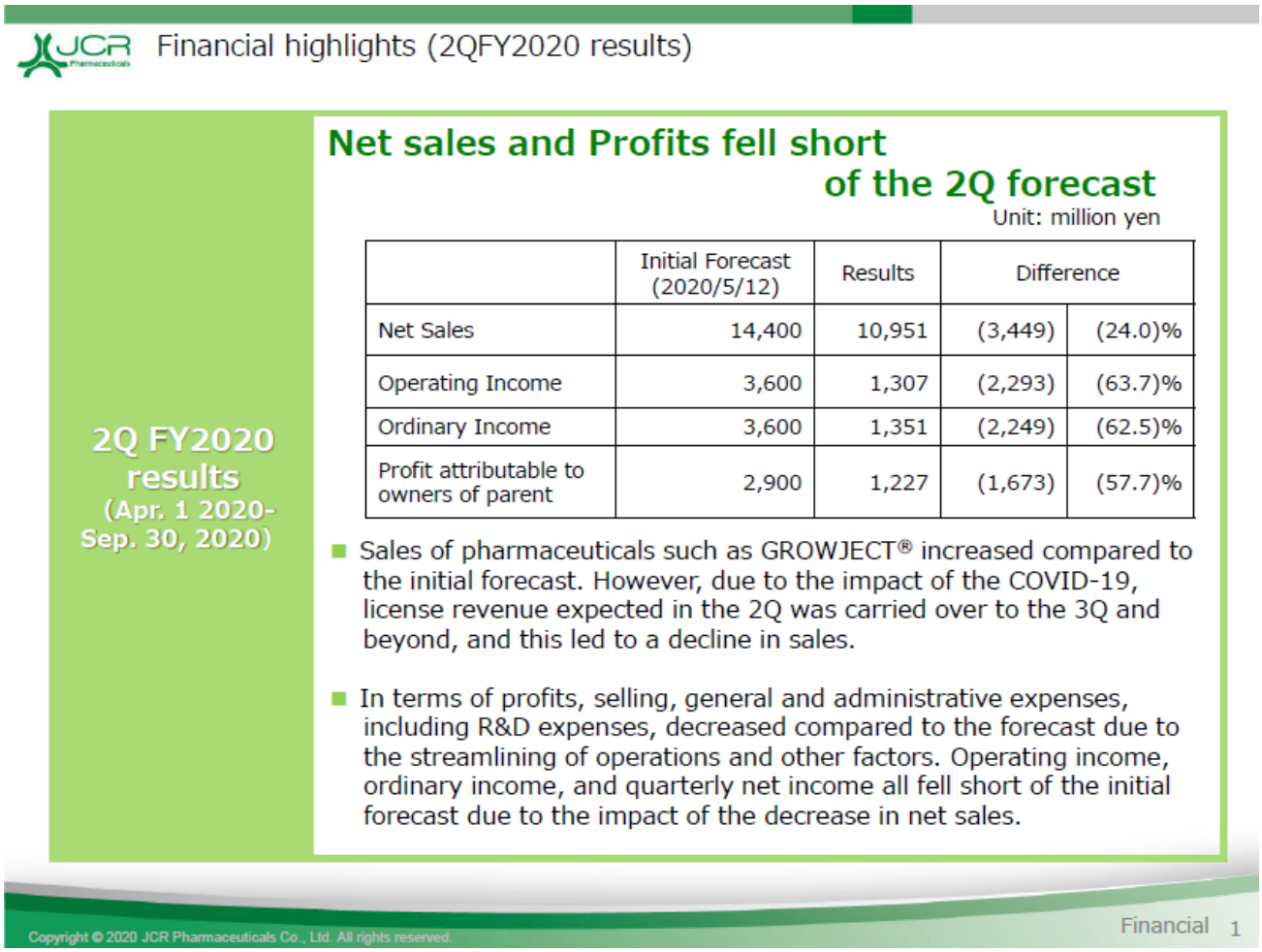
Next, I would like to talk about the discussions with AstraZeneca regarding domestic production of COVID-19 vaccine Bulk. As we have already announced, we are discussing the domestic contract manufacturing of the coronavirus vaccine bulk currently under development by AstraZeneca, which uses an adenoviral vector.

Regarding production of this vaccine bulk, it seems that AstraZeneca recognizes the high levels of skill that our Company possesses in the areas of biopharmaceutical manufacturing and genetic therapy. Using these technologies, we are making concerted efforts throughout the Company to ensure a stable supply of this vaccine in Japan as quickly as possible. When an official decision has been reached, we will rapidly begin production of the vaccine material.

In order to supply this vaccine, we also plan to work with Medipal Holdings, which is able to provide the social infrastructure support necessary for the stable supply of pharmaceutical products. Economic activities, due to the impact of the COVID-19 pandemic, are moving toward recovery; but the threat of the spread of infection has not yet resolved. We will strive to bring about an early end to this pandemic and continue to do what we can to contribute to people's health.

Due to the effect of COVID-19, in the first half of this fiscal year, several unexpected events have taken place. Shifts in the timing of licensing agreements and discussions over production of vaccine bulk, were both impossible to predict in advance. However, all R&D activities, including development of lysosomal disease treatment, have been steady. We are working to enhance our R&D structure to further promote the

development of gene therapies for the future. We are also considering expanding our manufacturing facilities, which will be responsible for the commercial production of new drugs in the future. I would like to ask for the continued understanding and support of our investors.



Haguchi: This is Akihiro Haguchi, Head of the Administrative Division. I would like to report on the summary of Results for the Second Quarter of the Fiscal Year Ending March 2021. First, please refer to the comparison with the initial forecasts.

Net sales decreased 24% compared to the initial forecast. As a result, we recorded sales of JPY10.95 billion. This was mainly due to the fact that although sales of pharmaceuticals, such as GROWJCET® and TEMCELL saw higher sales than were initially forecast, contract revenue that was due in the second quarter has been carried forward to the third quarter or later. This is due to the difficulty of conducting face-to-face negotiations as a result of the COVID-19 pandemic.

In terms of profits, selling, general and administrative expenses, including R&D expenses, decreased compared to the forecast due to the streamlining of operations and other factors. Operating income, ordinary income, and quarterly net income all fell short of the initial forecast due to the impact of the decrease in net sales.

Development of JR-141 and other treatments for lysosome diseases is progressing well. Regarding out-licensing agreements, as agreements are expected during the fiscal year under review, the Company has not revised its full-year earnings forecasts.

Consolidated Results

Consolidated	Six Months Ended Sep. 30, 2019	Six Months Ended Sep. 30, 2020 A	Year-on-Year	FY2020 (Apr. 1, 2020- Mar. 31, 2021) Initial Forecast B	Expected progress rate A/B
Net Sales	11,236	**10,951**	**(2.5)%**	27,200	40.3%
Cost of Sales	3,173	**3,513**	**10.7%**	6,800	51.7%
Gross Profit	8,063	**7,438**	**(7.8)%**	20,400	36.5%
SG&A	3,809	**3,723**	**(2.3)%**	8,000	46.5%
R&D Expenses	3,255	**2,407**	**(26.0)%**	6,400	37.6%
Operating Income	998	**1,307**	**31.0%**	6,000	21.8%
Ordinary Income	981	**1,351**	**37.7%**	6,000	22.5%
Profit*	922	**1,227**	**33.0%**	4,800	25.6%

*Profit attributable to owners of parent

(Reference)

R&D Exepenses**	3,539	**2,607**	**(26.3)%**	7,600	34.3%

**R&D Expenses before deducting contribution amount by collaborative R&D partners

Next, we will look at a comparison with the previous fiscal year. On this page, the figures for the second quarter and the YoY comparison are shown from the center to the left, and progress against the full-year forecast is shown on the right.

Net sales decreased 24% compared to the initial forecast, but only 2.5% compared to the previous fiscal year. The cost of sales ratio rose 3.5%, to 32.1%, mainly due to a decrease in contract revenue. Gross profit decreased 7.8%, to JPY7.4 billion.

On the other hand, selling general and administrative expenses declined 2.3% to JPY3.7 billion, as a result of efforts to improve operational efficiency, including responses to the coronavirus pandemic. R&D expenses declined 26% to JPY2.4 billion in the fiscal year under review, as payments to ArmaGen were recorded in the previous fiscal year.

Although net sales declined, we were able to secure an increase of more than 30% in operating income, ordinary income, and quarterly net income. As I mentioned earlier, there is no change to the full-year forecast.

Sales by business Segments (Consolidated)

(Unit: Million yen)

	Six Months Ended Sep. 30, 2019		Six Months Ended Sep. 30 2020			FY2020 (Apr. 1, 2020- Mar. 31, 2021)	
		Composition ratio	A	Composition ratio	Year-on-Year	Forecast B	Expected progress rate A/B
GROWJECT®	6,235	55.5%	**6,538**	**59.7%**	**4.9%**	**13,270**	**49.3%**
Treatment for renal anemia	2,272	20.3%	**3,390**	**31.0%**	**49.2%**	**6,540**	**51.8%**
Epoetin Alpha BS Inj.[JCR]	2,272	20.3%	1,696	15.5%	(25.4)%	3,350	50.6%
Darbepoetin Alpha BS Inj.[JCR]	—	—	1,694	15.5%	—	3,190	53.1%
TEMCELL®HS Inj.	1,527	13.6%	**784**	**7.2%**	**(48.6)%**	**2,110**	**37.2%**
Agalsidase Beta BS I.V. Infusion [JCR]	123	1.1%	**220**	**2.0%**	**79.1%**	**1,060**	**20.8%**
Other products	74	0.6%	**7**	**0.0%**	**(90.5)%**	**0**	—
Total Pharmaceuticals, etc.	10,234	91.1%	**10,941**	**99.9%**	**6.9%**	**22,980**	**47.6%**
Lincense Revenue	1,002	8.9%	**10**	**0.1%**	**(99.0)%**	**4,220**	**0.2%**
Total Net Sales	11,236	100%	**10,951**	**100.0%**	**(2.5)%**	**27,200**	**40.3%**

Next, we will see a breakdown of net sales. On this page, with regard to the breakdown of net sales, the comparison with the previous year is shown from the center to the left, and the progress against the full-year forecast is shown on the right.

Although there were NHI drug price revisions in April of this year, due to an increase in sales volume, sales of GROWJECT®, the growth hormone formulation, treatment for renal anemia and Agalsidase Beta BS, a treatment for Fabry disease, both increased compared with the previous fiscal year.

With regard to drugs for the treatment for renal anemia, sales of Darbepoetin Alfa BS, which was launched in November last year, were favorable. As a result, total sales with Epoetin Alfa BS increased 49% compared with the previous fiscal year.

Revenue for TEMCELL® was down 48% due to the effects of supply restrictions implemented to ensure inventory. However, total sales of TEMCELL® products increased 6.9% from the previous period to JPY10.94 billion.

On the other hand, as I mentioned at the beginning, contract revenue, which had been anticipated in the second quarter, has been carried over to the third quarter or later. As a result, total net sales were JPY10.95 billion, down 2.5% YoY.

Balance Sheets (Consolidated)

(Unit: Million yen)

	Mar. 2020	Sep. 2020	Main Increase/decrease		Mar. 2020	Sep. 2020	Main Increase/decrease
Current assets	28,342	**37,504**	Cash and deposit +8,937 Accounts receivable (1,462) inventory +1,562	**Current liabilities**	10,434	**20,755**	Short-term borrowings +8,720 Income taxes payable +504
				Non-current liabilities	4,761	**5,480**	Borrowings and bonds +700
Non-current assets	19,433	**22,204**	Property, plant and equipment (474) Patent right +3,126	**Total liabilities**	15,195	**26,236**	+11,040
				Total net assets	32,579	33,472	Profit +1,227 Dividend (525)
Total	47,775	**59,708**	+11,932	**Total**	47,775	**59,708**	+11,932

Capital investment	5,296	**306**	**Equity ratio**	66.6%	**54.8%**	

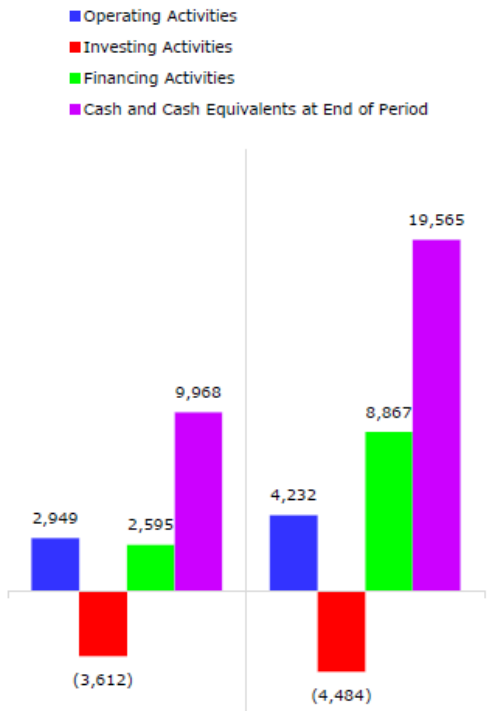
Next, the balance sheet. There are two main themes in the second quarter of this fiscal year.

First, we have accumulated cash on hand in response to the COVID-19 pandemic. As a result, cash and deposits and borrowings each increased by approximately JPY9 billion. To date, the only effect of coronavirus has been on lysosomal disease drug negotiations. Other areas, such as production, research, and sales have not been adversely affected. However, amid increasing uncertainty about the outlook for the global economy, and with our focus on global expansion, we have increased our cash on hand.

The second point is the booking of patent rights. In conjunction with the consolidation of ArmaGen in April of this year, JPY3.1 billion of patents have been recorded. As a result, total assets increased by JPY11.9 billion from the previous fiscal year-end to JPY59.7 billion. The equity ratio declined 11.8 percentage points to 54.8%.

Cash Flows (Consolidated)

(Unit: Million yen)



	Six Months Ended Sep. 30, 2019 A	**Six Months Ended Sep. 30, 2020 B**	Year-on-year B - A
Income before income taxes	1,098	1,381	283
Depreciation and amortization	658	872	213
Accounts receivable-trade	2,098	1,462	(636)
Inventories	(860)	(1,562)	(702)
Other	(45)	2,078	2,124
Operating Activites	2,949	**4,232**	1,283
Securities	239	0	(239)
Capital investment	(3,852)	(1,437)	2,415
Other	0	(3,047)	(3,047)
Investing Activities	(3,612)	**(4,484)**	(871)
Borrowings	3,200	9,420	6,220
Dividends·treasury stock	(515)	(516)	(1)
Other	(89)	(36)	53
Financing Activities	2,595	**8,867**	6,272
Cash and Cash Equivalents at End of Period	9,968	**19,565**	9,596

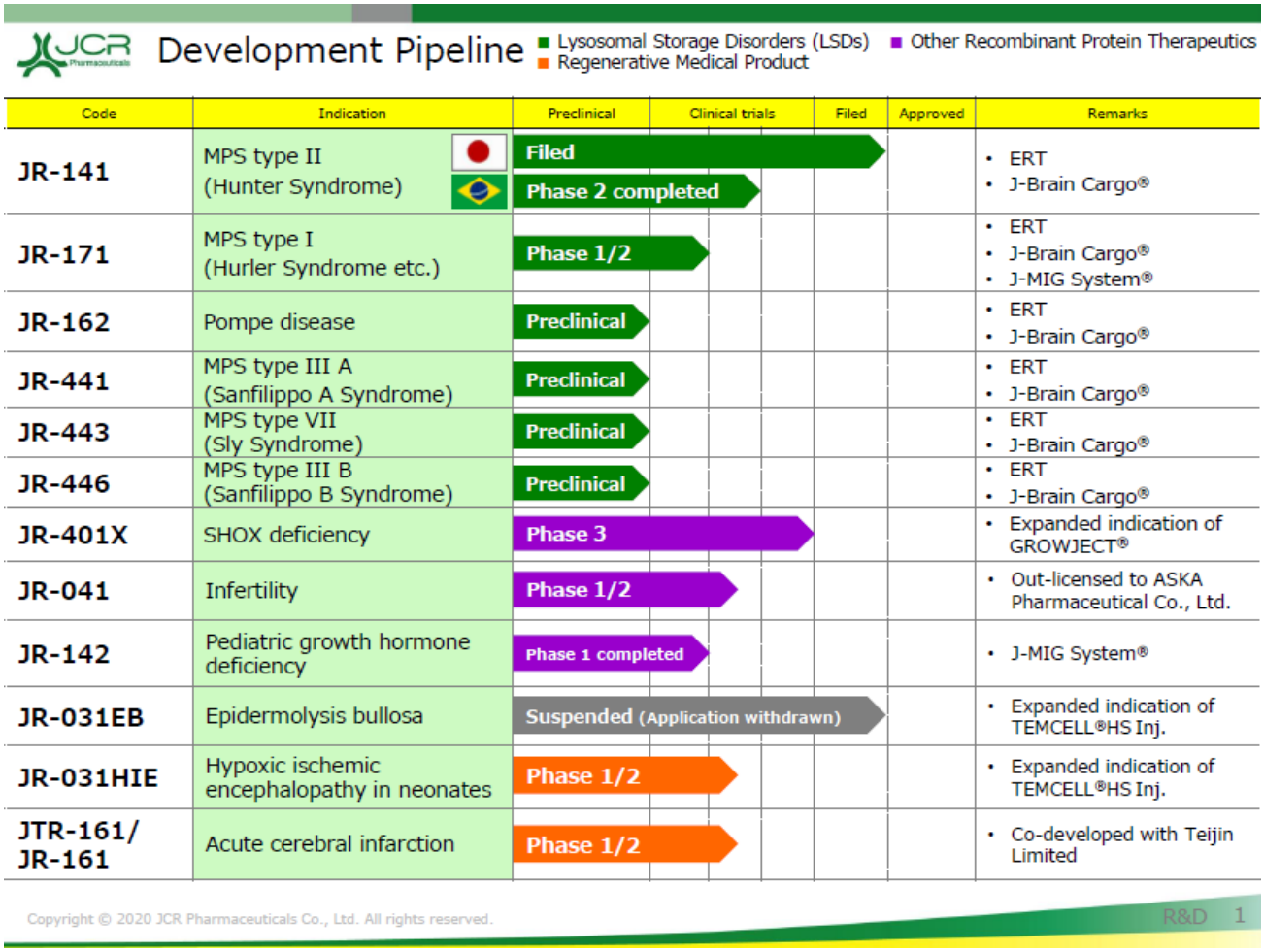
Finally, cash flow.

Net cash provided by operating activities was JPY4.2 billion, an increase of JPY1.2 billion from the previous fiscal year.

Net cash used in investing activities amounted to JPY4.4 billion due to capital expenditures and the acquisition of patent rights.

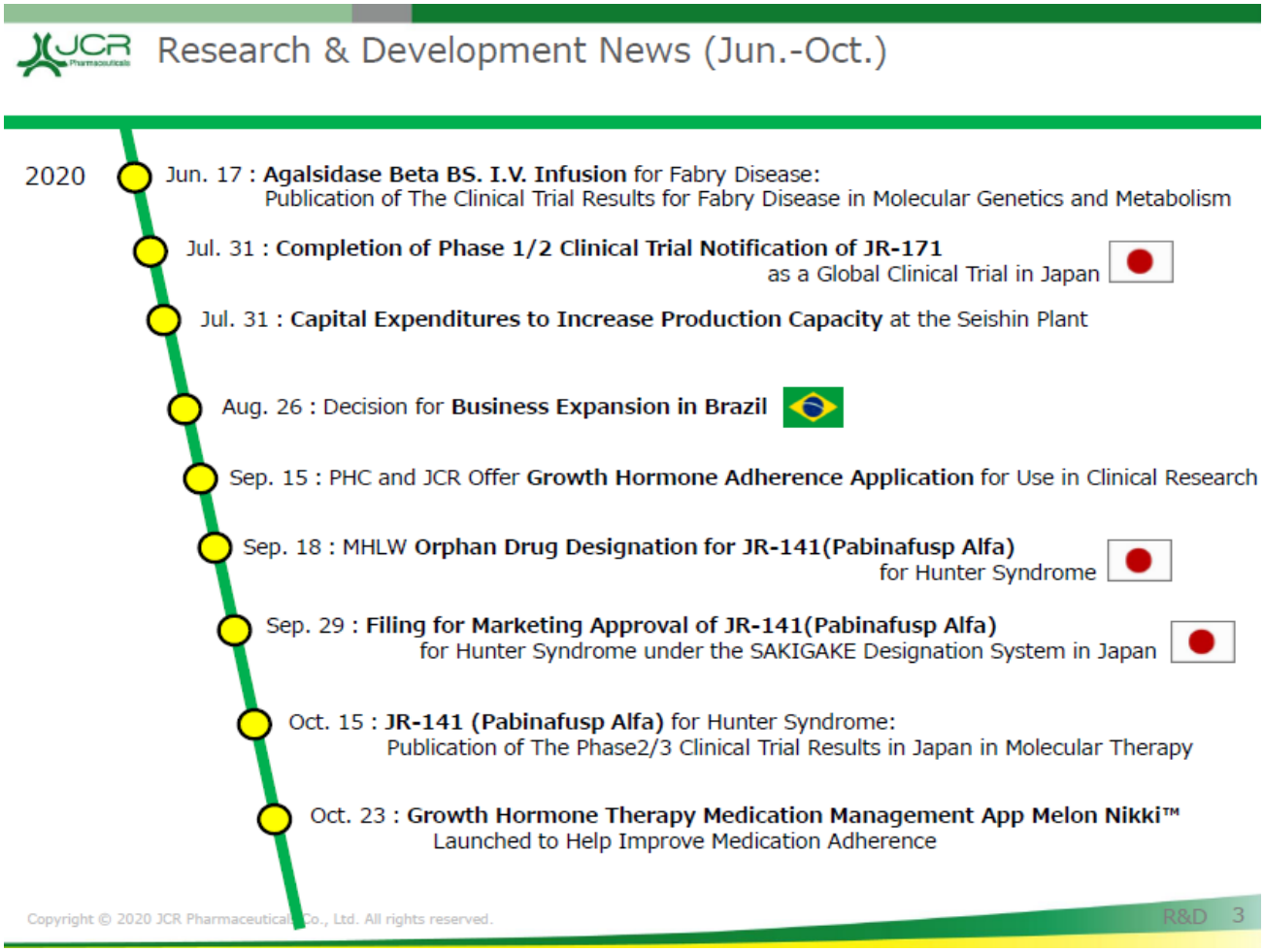
Cash flows from financing activities, on the other hand, resulted in an inflow of JPY8.8 billion as the Company borrowed funds to increase cash on hand.

As a result, cash and cash equivalents at the end of September were JPY19.5 billion, an increase of approximately JPY9.6 billion from the end of the previous fiscal year. Thank you very much for your attention.

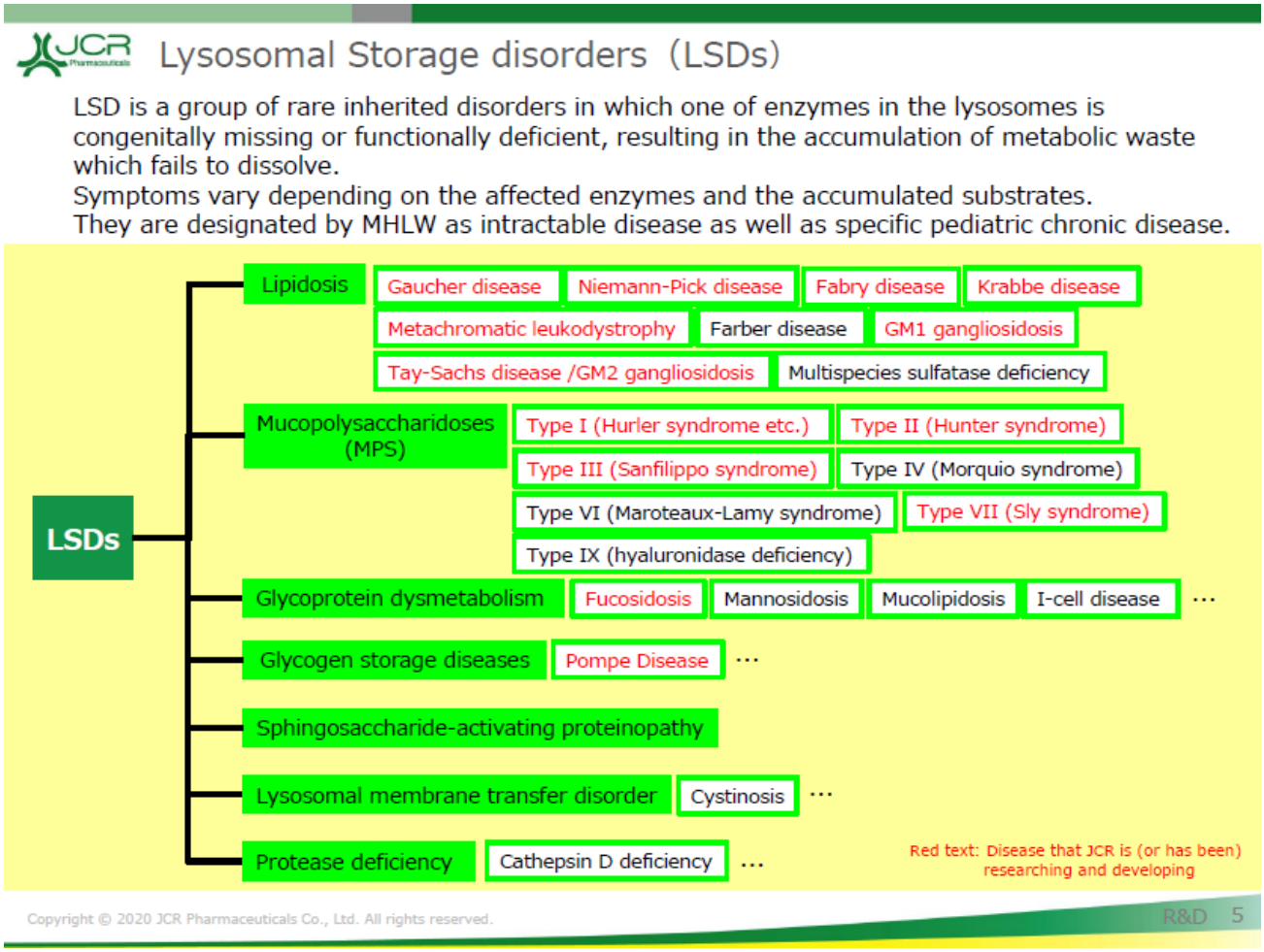


Tanizawa: This is Kazunori Tanizawa of the Development Division. I would like to report on the progress of R&D.

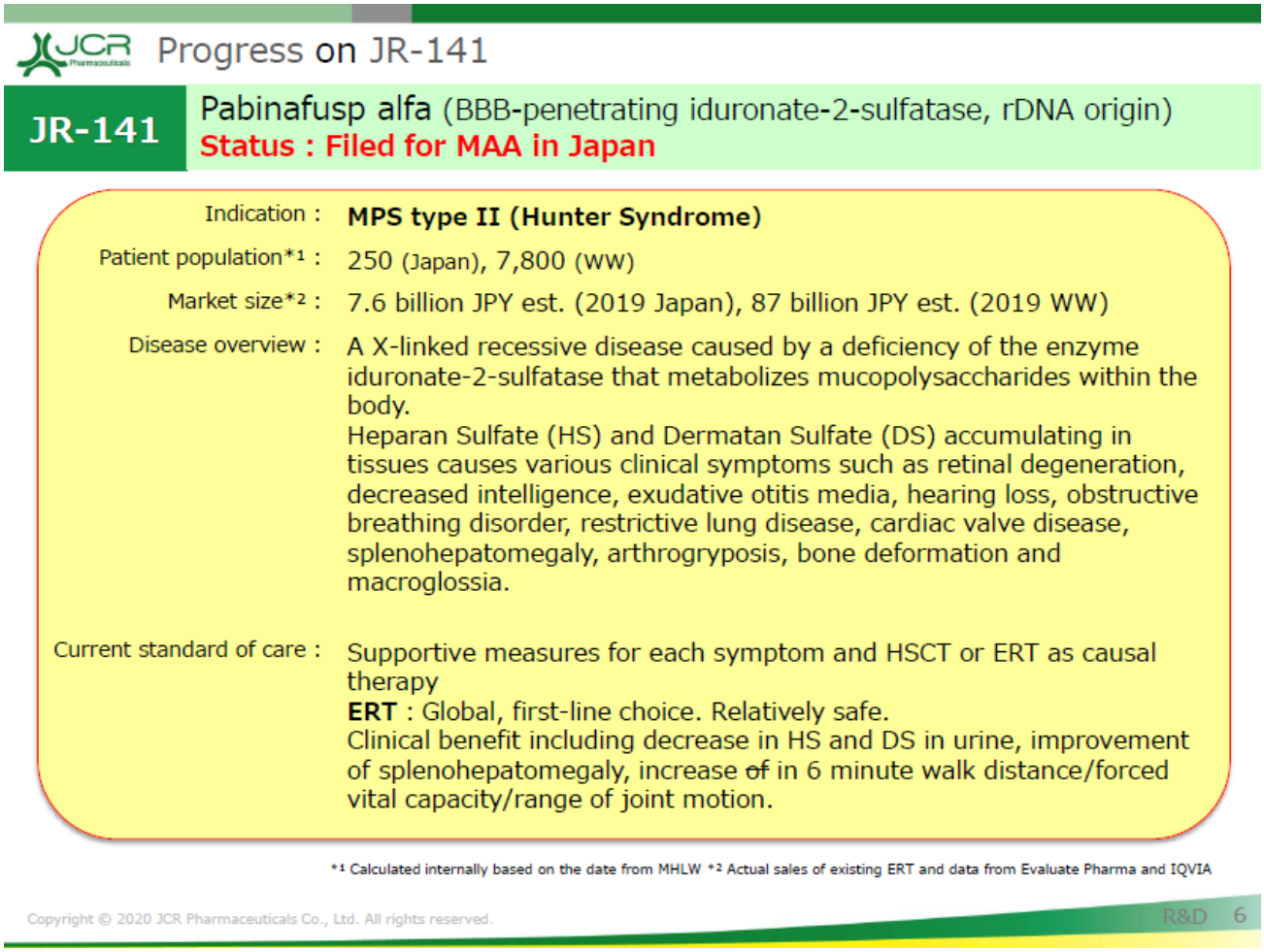
This is the development pipeline. Green colors indicate the lysosomal disease treatments, purple colors indicate protein preparations, and orange colors indicate regenerative medical products.



The highlights of the news releases for R&D are shown. On July 31, the Phase 1/2 Japan clinical trial for JR-171 was accepted. In August, we officially decided to expand our business into Brazil. On the 29th of September, we submitted an application for marketing authorization for JR-141.

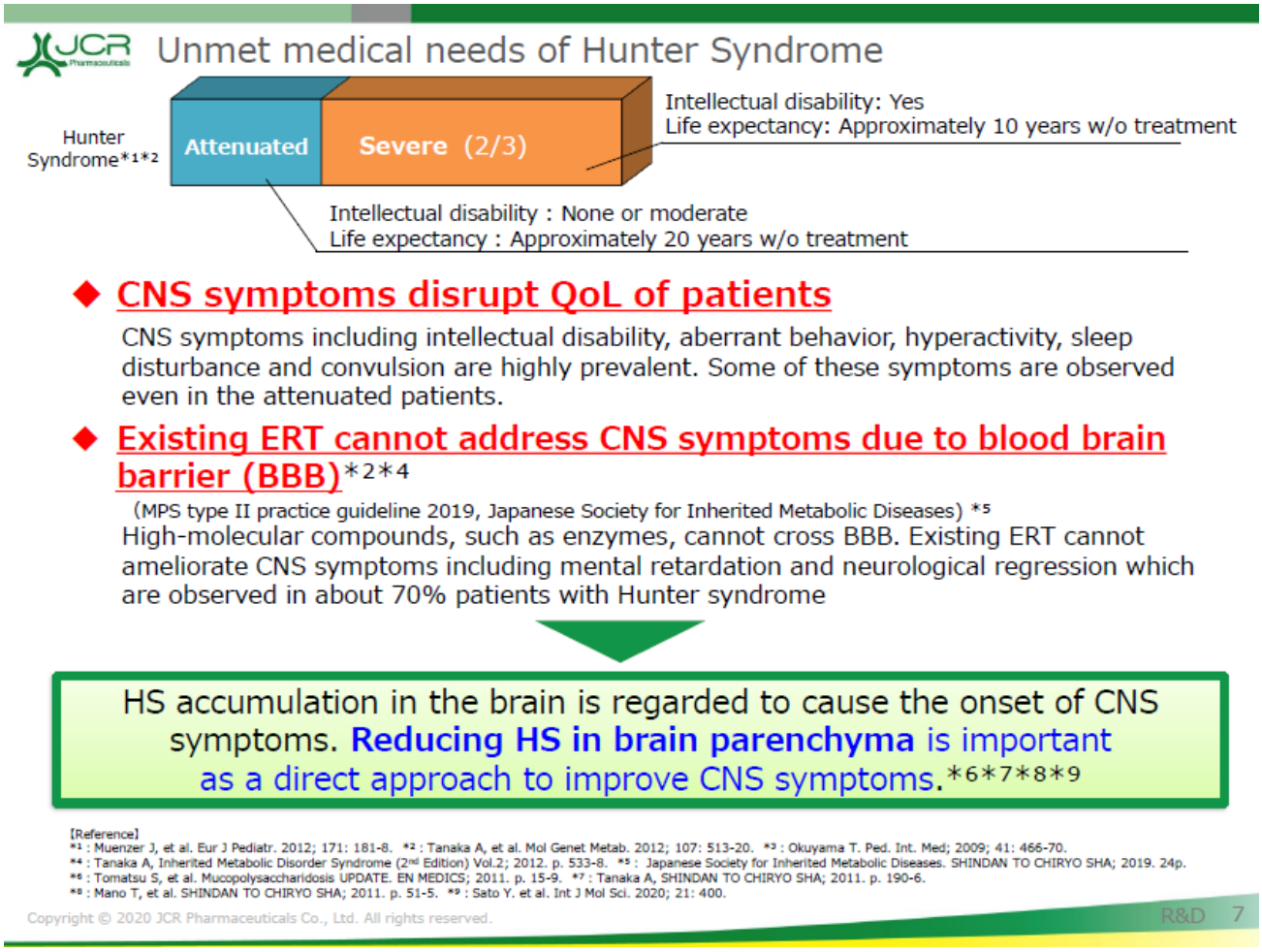


Here, I would like to explain the status of JR-141 development. Of the lysosomal diseases shown here, those shown in red are the diseases currently being tackled by JCR.



Of these, we are currently developing JR-141 for mucopolysaccharidosis type II, or Hunter Syndrome. Mucopolysaccharidosis type II is an X-linked recessive disorder caused by a deficiency in iduronate-2-sulfatase, as shown here.

Heparan sulfate and dermatan sulfate accumulate in bodily tissues, including those of the central nervous system leading to a variety of clinical conditions.



In Hunter Syndrome, two-thirds of patients are said to have intellectual disability. These patients with central nervous system disease have a significantly reduced QOL. The lack of current treatment methods is a major unmet medical need, and JCR is working to resolve this issue through the development of JR-141.

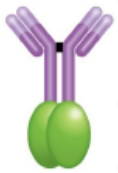
To do this, we have focused our attention on reducing the levels of accumulated heparan sulfate in the brain tissue. The rationale is that this direct approach will reduce, will lead to improvement in central nervous system symptoms.

JR-141

JR-141 Pabinafusp alfa (BBB-penetrating iduronate-2-sulfatase, rDNA origin)
Status : Filed for MAA in Japan

JR-141 Summary and Estimated delivery route of J-Brain Cargo®

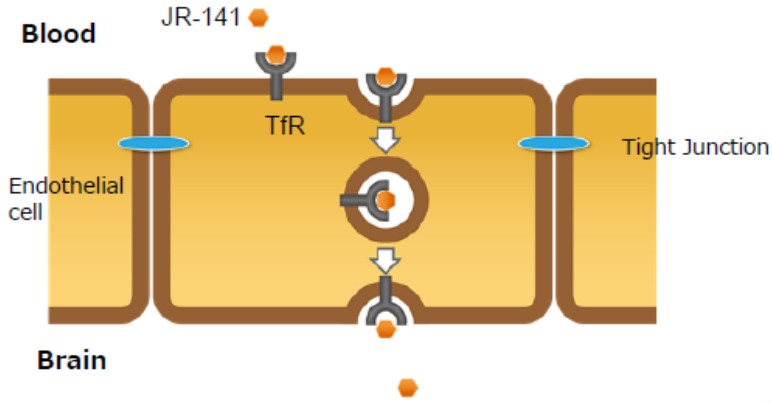
<JR-141>



J-Brain Cargo®
(Anti human-Transferrin Receptor antibody)

hIDS (2 molecules)

- Recombinant fusion protein of humanized antibody (J-Brain Cargo®) specifically binding human transferrin receptor (TfR) and human Iduronate-2-Sulfatase (hIDS)
- Produced by CHO
- Intravenously infusion, Lyophilized.

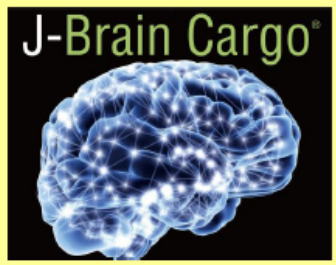


The international nonpropiretary name for JR-141 is Pabinafusp alfa. JR-141 consists of a J-Brain Cargo® component, which is an anti-human transferrin antibody, and two IDS enzyme molecules.

JR-141 binds to the transferrin receptors expressed on the endodermal cells lining cerebral capillaries. They then move through the cells and into the brain tissue crossing the blood-brain barrier.

History of JR-141 development

2005	• **Initiation of BBB-penetrating technology research**
(2007)	(ERT for Hunter syndrome launched in Japan)
2014	• **Initiation of JR-141 development**
	• Initiation of non-clinical testing
	• **Confirmation of BBB penetration into CNS** (Sonoda H, et al. Mol Ther. 2018; 26(5):1366-74.)
	• **Suppression of CNS symptom in MPSII mouse** (manuscript submitted)
2017	• Initiation of Phase 1/2 clinical trial in Japan
	• **Indicating clinical significance of J-Brain Cargo®** (Okuyama T, et al. Mol Ther. 2019; 27(2):456-64.)
2018	• Designation under **SAKIGAKE system** in Japan
	• Initiation of Phase 2 trial in Brazil
	• Initiation of Phase 2/3 trial in Japan (Okuyama T, et al. Mol Ther. 2020; 2020 Sep 30;S1525-0016(20)30496-2)
	• Orphan drug designation in U.S.
2019	• Orphan drug designation in EU.
2020	• Orphan drug designation in Japan.
	• **Application for marketing authorization in Japan**

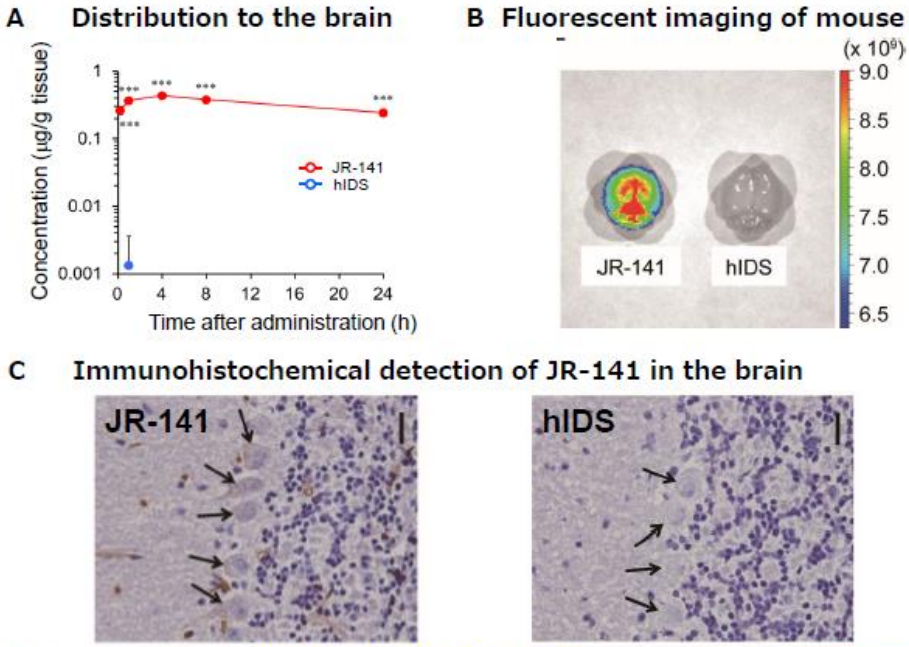


The development of JR-141 has been ongoing for 15 years. However, as you can see, we started Phase 1/2 trial in 2017. We recognize that we are now able to file for marketing approval in 2020, which is a very short development period. We recognize that this is the result of the extremely high expectations of patients and doctors.

JR-141 non-clinical test

JR-141 Pabinafusp alfa (BBB-penetrating iduronate-2-sulfatase, rDNA origin)
Status : Filed for MAA in Japan

- Incorporation of JR-141 to neuronal tissues in of the brain following penetration via BBB intravenous administration into hTfR-KI Mice



Sonoda et al., Mol Ther. 2018; 26: 1366-1374(DOI: https://doi.org/10.1016/j.ymthe.2018.02.032)

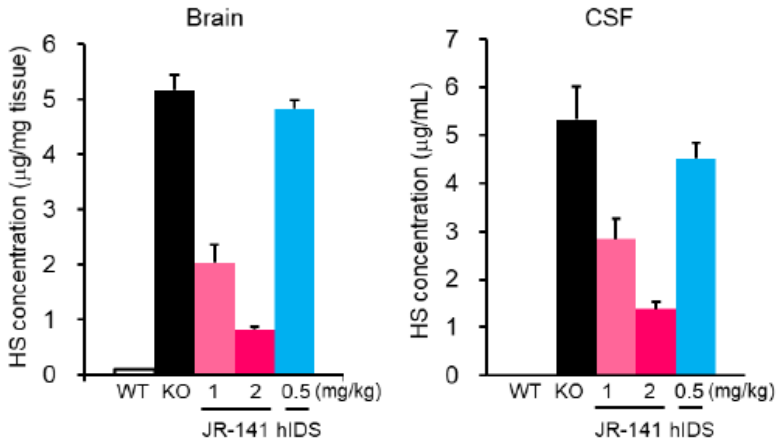
R&D 10

The results of non-clinical tests are shown here. As the details have been published, I will omit them here. However, it can be confirmed that JR-141 passes through the blood-brain barrier and is incorporated into the neuronal tissues of the brain.

JR-141 non-clinical test

JR-141 Pabinafusp alfa (BBB-penetrating iduronate-2-sulfatase, rDNA origin)
Status : Filed for MAA in Japan

Effect on HS accumulation in MPS II mice



- JR-141 decreases HS in brain and Cerebral spinal Fluid (CSF)
- A correlation between HS in brain tissues and CSF indicates utility of CSF HS as a biomarker to measure HS levels in the brain

Tanaka et al., Mol Genet Metab. 2018; 125: 53-58 (DOI: https://doi.org/10.1016/j.ymgme.2018.07.013)

In this case, a decrease in heparan sulfate was confirmed in knockout mice. The effect is visible, both in the brain, and in the CSF. As you can see, there is a dose-dependent decrease in heparan sulfate.

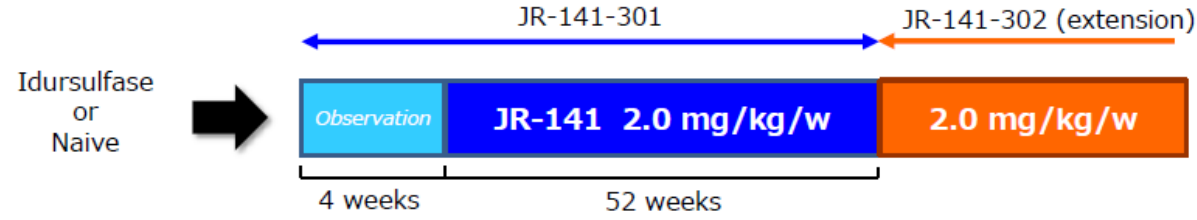
In addition, we can see that the decrease of substrate in the brain, and the decrease of substrate in the CSF, are very well correlated.

What this data implies is that the decline in heparan sulfate in CSF, which has been confirmed in clinical trials, represents a decline in the accumulation in the brain.

JR-141 Clinical trial in Japan

JR-141 Pabinafusp alfa (BBB-penetrating iduronate-2-sulfatase, rDNA origin)
Status : Filed for MAA in Japan

Phase 3 trial (JR-141-301) : Brief Summary



Primary endpoint	Heparan Sulfate (HS) in CSF
Secondary endpoint	• Developmental evaluation (cognitive, adoptive behavior) • Dermatan Sulfate (DS) reduction in CSF • HS and DS reduction in blood and urine • Liver volume, spleen volume • 6 minute walk distance • Joint range of motion
Number of subjects	28 (Target number of subjects: 20)
Manuscript	Okuyama T, et al. DOI: https://doi.org/10.1016/j.ymthe.2020.09.039

Phase 3 trial have already been completed, and many patients are currently receiving JR-141 in the ongoing 302 trial.

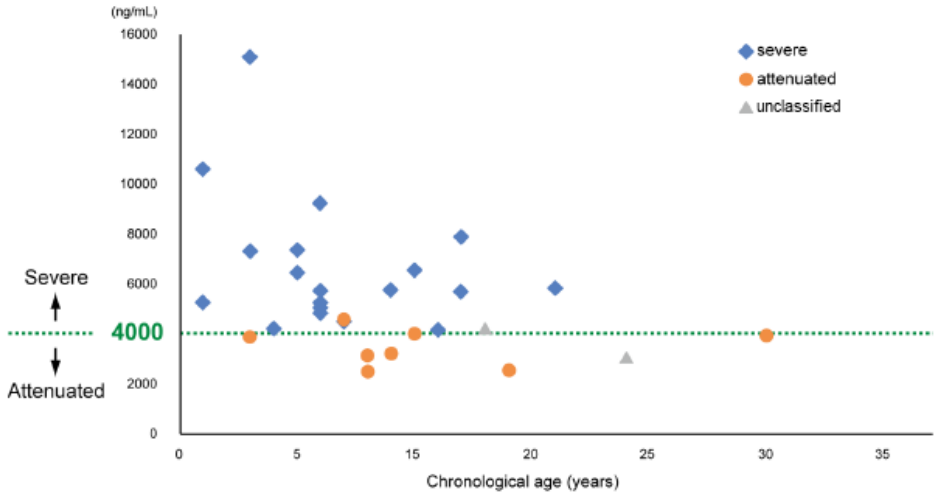
The primary endpoint is cerebrospinal fluid, heparan sulfate concentration. Secondary endpoints include developmental evaluation, blood and urine heparan sulfate, and dermatan sulfate concentrations, and six-minute walk distance. Twenty-eight patients from 19 facilities nationwide participated.

JR-141 Clinical trial in Japan

JR-141 Pabinafusp alfa (BBB-penetrating iduronate-2-sulfatase, rDNA origin)
Status : Filed for MAA in Japan

Phase 3 trial (JR-141-301) : Results

Individual CSF HS Levels in Trial JR-141-301 at Baseline



Findings
- The CSF HS concentration is a biomarker that correlates well with disease severity

First, this graph shows CSF heparan sulfate concentration at the start of the study.

As you can see in this figure, we show baseline data for patients with orange dots, mild symptoms, and blue dots, severe symptoms.

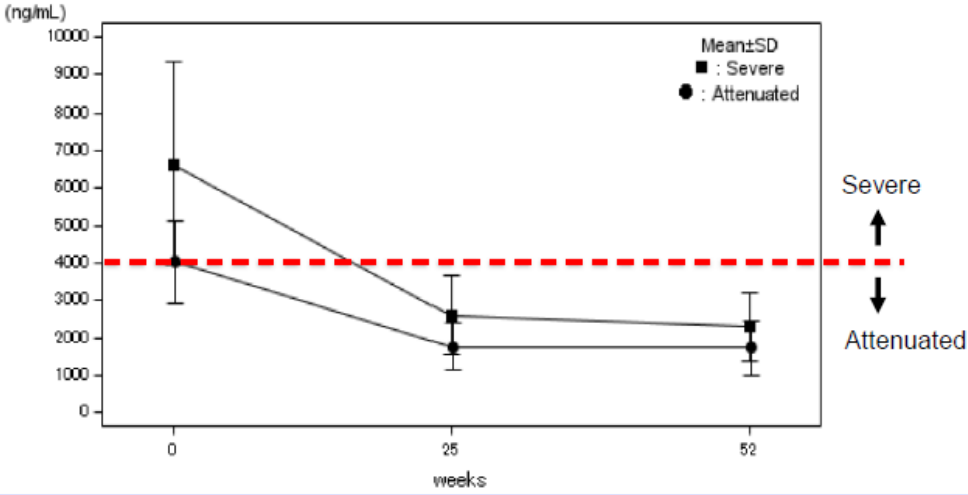
As you can see, the orange dots are gathered in the lower part, and there is a cut-off value that separates the attenuated type from the severe type. The cut-off figure is approximately 4,000 nanograms per milliliter. In this study, we demonstrated that heparan sulfate in CSF correlates well with disease severity.

JR-141 Clinical trial in Japan

JR-141 Pabinafusp alfa (BBB-penetrating iduronate-2-sulfatase, rDNA origin)
Status : Filed for MAA in Japan

Phase 3 trial (JR-141-301) : Results

Time course of HS reduction in CSF



Findings
- CSF levels decreased in almost all patients to a level comparable with attenuated patients

Next, we show how the value has changed after treatment. Squares represent the severe type, and circles represent the attenuated type.

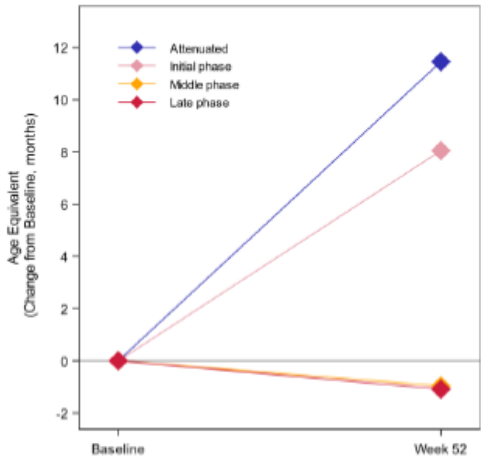
In both cases, the concentration of heparan sulfate declined at 25 and 52 weeks, eventually falling to around the 2,000 level. I think this can be said to be a sufficiently attenuated type level.

JR-141 Clinical trial in Japan

JR-141 Pabinafusp alfa (BBB-penetrating iduronate-2-sulfatase, rDNA origin)
Status : Filed for MAA in Japan

Phase 3 trial (JR-141-301) : Results

Mean Changes in Age Equivalent Score (AES) observed in various Disease Severity Groups



Initial phase : <3y and <80 of Development Quotient
Middle phase : ≤8y or ≥20 of Development Quotient
Late phase : >8y or ≤20 of Development Quotient

Disease phenotype	No Subjects	Slope
Attenuated	8	0.9543
Severe: initial phase	2	0.6705
Severe: middle phase	11	-0.0802
Severe late phase	5	-0.0904

Findings:
- Increases in AES from baseline were observed in attenuated and severe patients in the initial phase
- Stabilization of AES was observed in patients in the middle and late disease phase

In this study, we are also evaluating age equivalent score. In the chart on the left, the blue line at the top represents patients with attenuated type. As a result of treatment for 52 weeks, an increase in the age of development close to 12 months has been recognized, and the result is that normal development has been recognized.

The line below that is the data for severe patients under three years of age. As can be seen, an increase in the age of development was recognized after 12 months of treatment.

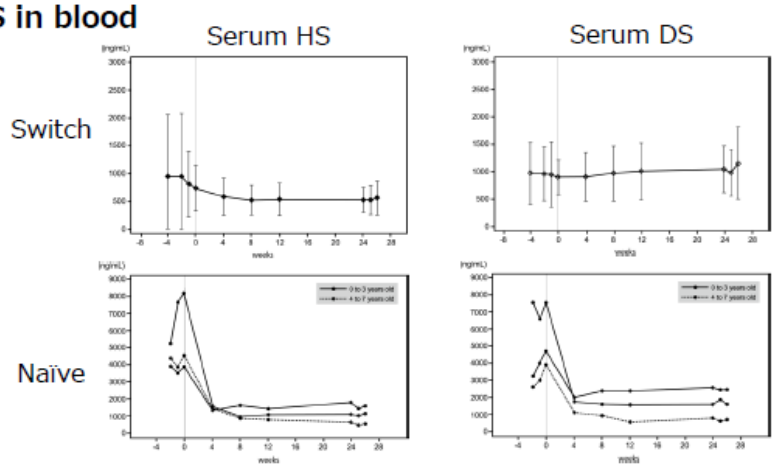
Those aged three years or older are characterized by a reduced developmental age at the start of the study. In this group, developmental age is maintained over the treatment period.

JR-141 Clinical trial in Japan

JR-141 Pabinafusp alfa (BBB-penetrating iduronate-2-sulfatase, rDNA origin)
Status : Filed for MAA in Japan

Phase 3 trial (JR-141-301) : Results

HS and DS in blood



Findings:
- Serum HS and DS remained stable in patients switched from IDS to JR-141
- Serum HS and DS rapidly decreased in ERT-naïve patients treated with JR-141
- These results including other secondary endpoints indicate that JR-141 has comparable efficacy on systemic symptom to existing ERT

Since JR-141 is an enzyme replacement therapy, it is extremely important to evaluate the whole body. We have shown here the changes in heparan sulfate and dermatan sulfate concentrations in the blood.

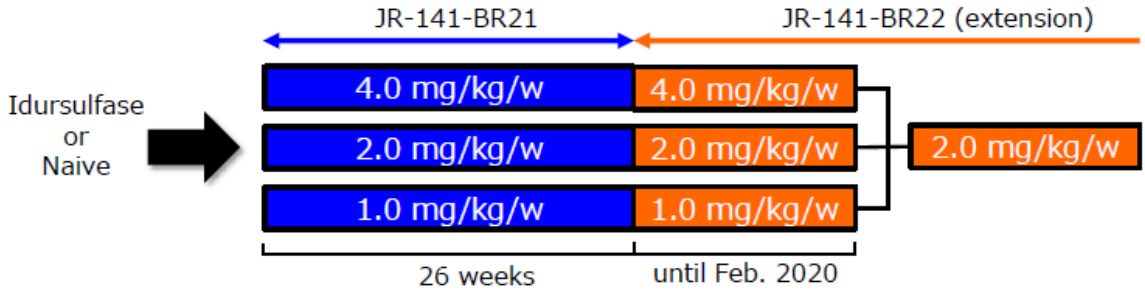
The graphs at the top show patients who have switched to 141. Those below show the data for patients who are treatment-naïve. As you can see, in the example of switching, you can see that both HS and DS in the blood are stable. As for new cases, the concentration rapidly decreased with administration and is maintained thereafter as expected.

In addition, we are reviewing secondary endpoints, and we believe that JR-141 is expected to be as effective as existing enzyme replenishment therapy for systemic symptom.

JR-141 Clinical trial in Brazil

JR-141 Pabinafusp alfa (BBB-penetrating iduronate-2-sulfatase, rDNA origin)
Status : Filed for MAA in Japan

Phase 2 trial (JR-141-BR21) : Brief Summary



Primary endpoint	Safety
Secondary and exploratory endpoints	• HS and DS concentration in CSF, serum and urine • Developmental evaluation (cognitive, adoptive behavior) etc.
Number of subjects	20 (Target number of subjects: 18)
Presentation	Oral and Poster presentations at the WORLD*Symposium* 2020

I would now like to move on to the results of trials in Brazil.

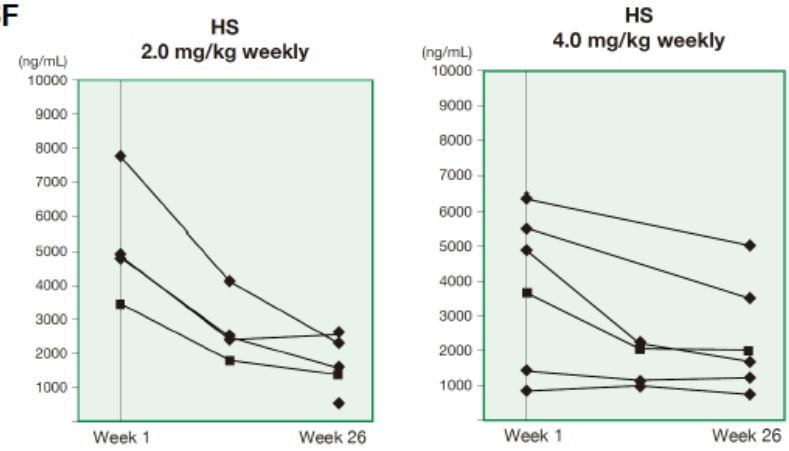
The study in Brazil was characterized by the examination of three doses: one, two and four milligrams per kilogram. This study also evaluates the developmental and evaluates biomarkers in cerebrospinal fluid and blood and urinalysis.

JR-141 Clinical trial in Brazil

JR-141 Pabinafusp alfa (BBB-penetrating iduronate-2-sulfatase, rDNA origin)
Status : Filed for MAA in Japan

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

HS in CSF



Findings
- Decrease of HS concentration in CSF in all subjects of the 2.0 mg and 4.0 mg treatment group

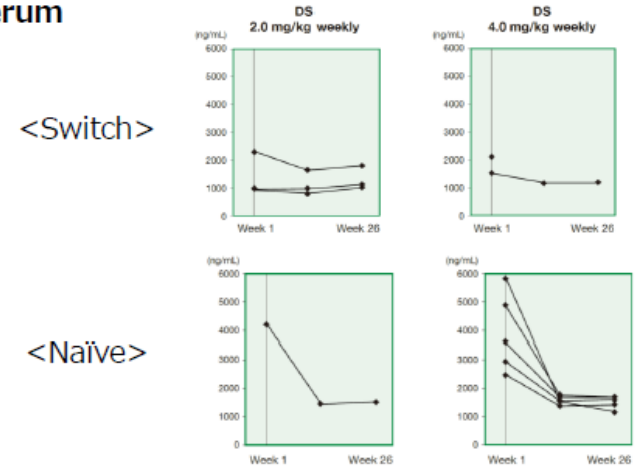
The results from the Brazil trial are similar to those from the Japan trial I presented earlier. The CSF heparan sulfate concentration decreased in all cases that received two milligram or four milligram doses.

JR-141 Clinical trial in Brazil

JR-141 Pabinafusp alfa (BBB-penetrating iduronate-2-sulfatase, rDNA origin)
Status : Filed for MAA in Japan

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

DS in serum

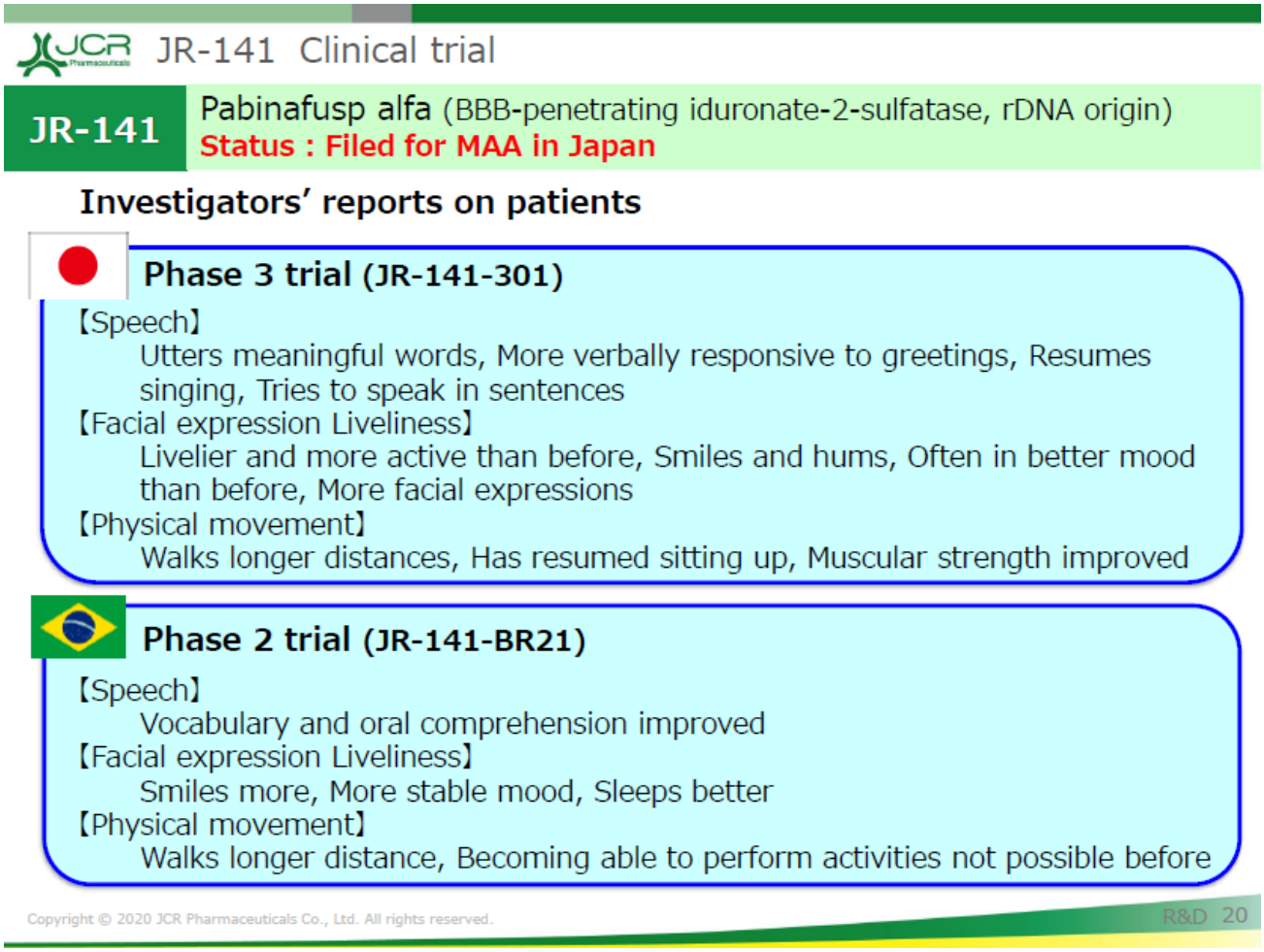


Findings:

- Serum DS remained stable in patients switched from IDS to JR-141
- Serum DS rapidly decreased in ERT-naïve patients treated with JR-141
- These results indicate that JR-141 has comparable efficacy to existing ERT in reducing serum DS

This shows the dermatan sulfate concentration in the blood.

It is known that dermatan sulfate is very closely related to systemic symptoms, so it is very important to look at this marker. As with the results in Japan, we confirmed stable in patients switching treatment, and decrease in treatment-naïve patients.



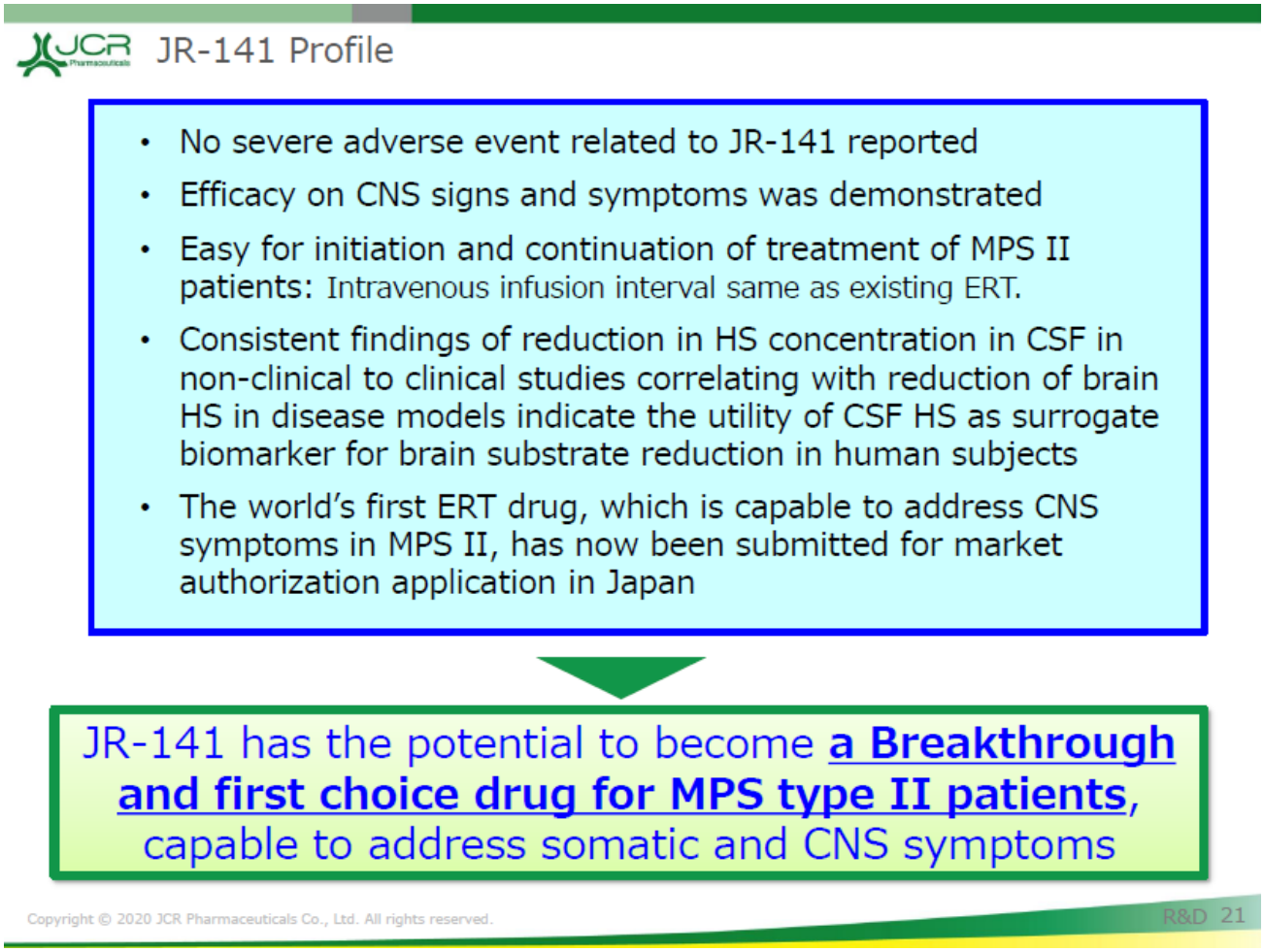
This slide is very important. Information on changes in subjects in both the Japanese study and the Brazilian study has been collected from the investigators in charge of the study and is summarized here.

As can be viewed, various improvements are recognized in terms of language, expressions, and movement. In particular, I would like to draw your attention to the linguistic aspect. I have heard that there has been increase in vocabulary, increase in comprehension, and improved ability to understand verbal instructions. In Brazil, communication in sentence has been reported.

However, these reports are subjective. In addition to this data, we also need objective indicators in order to discuss efficacy in clinical trials.

We are, therefore, using scores, such as the Bayley-III score, which we have implemented elsewhere, or the Vineland score to measure behavioral changes.

The results include improvements in the language and communication sub-domains of the Vineland score. So, we have also been able to demonstrate objective improvements in behavior.

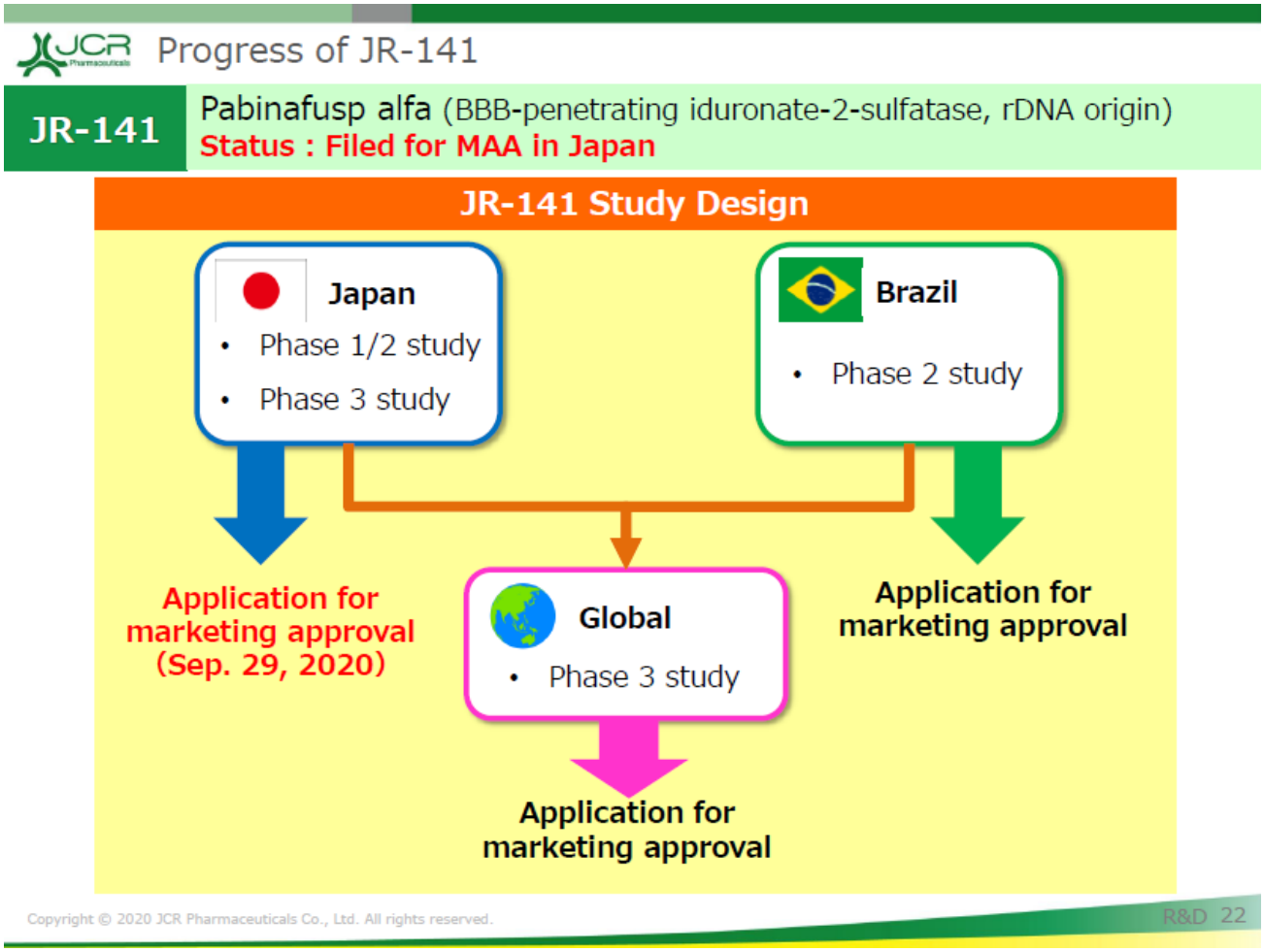


So far, I have summarized the details of the clinical trials that I have described. No severe adverse events related to JR-141 administration have been identified in previous studies. In addition, we believe that the efficacy against the central nervous system diseases and indications has been confirmed.

As the drug is intravenously administered, it is easy to switch from existing, similar treatments.

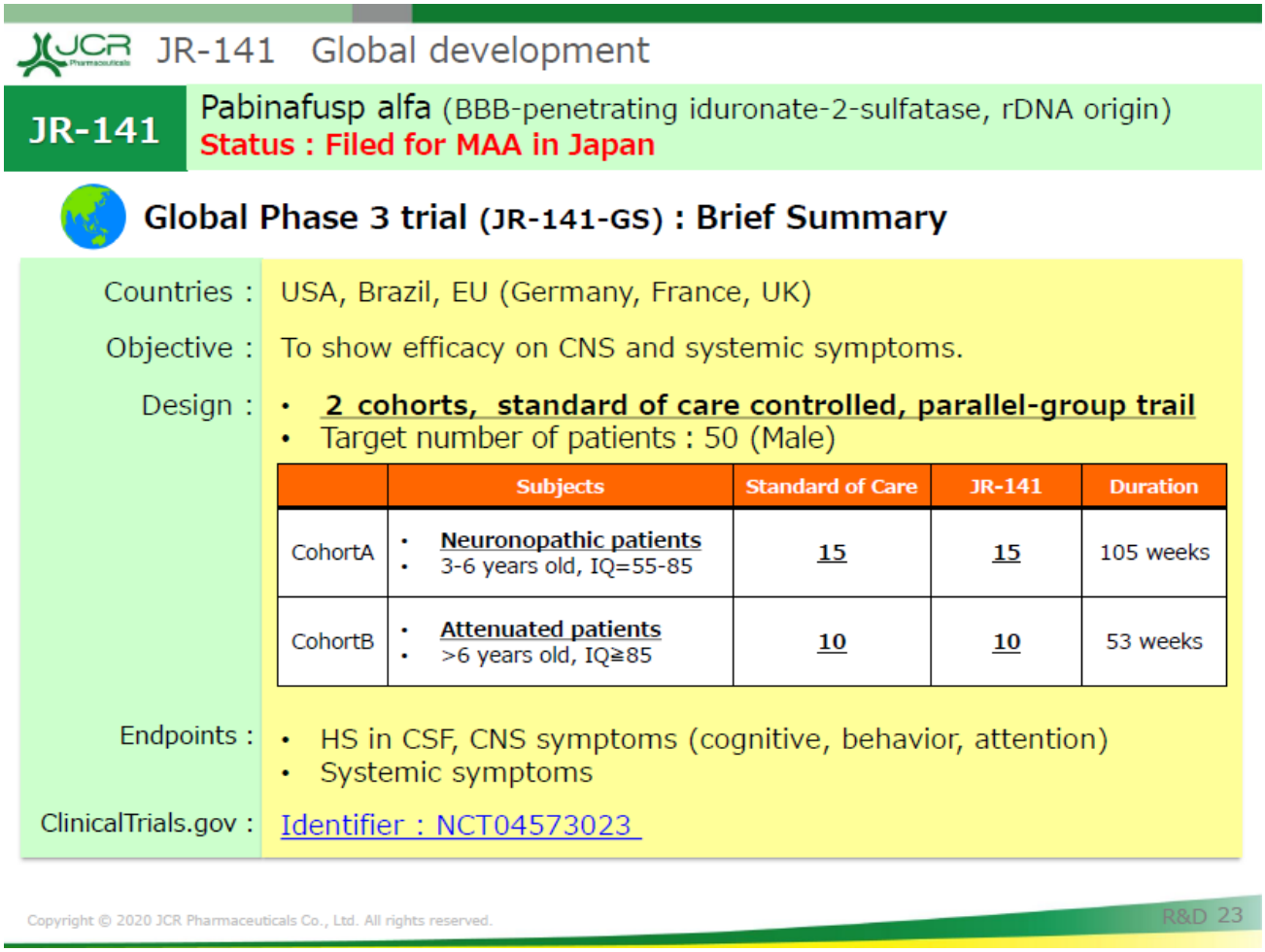
As we have consistently demonstrated from non-clinical trials, these clinical trials also seems to show the validity of measuring CSF heparan sulfate concentration as a surrogate for substrate concentration in brain tissue.

An application for the drug was submitted for marketing authorization in September. It has been designated as a pioneer, *sakigake*, treatment. So, if the application proceeds as expected, we expect approval to be obtained in March next year. I think this is a world first.



Now, let me explain the whole picture of the development situation.

Japan is as I just explained. For Brazil, we also plan to file an application for marketing approval this year. In parallel with this, we plan to conduct Phase 3 clinical trial globally.

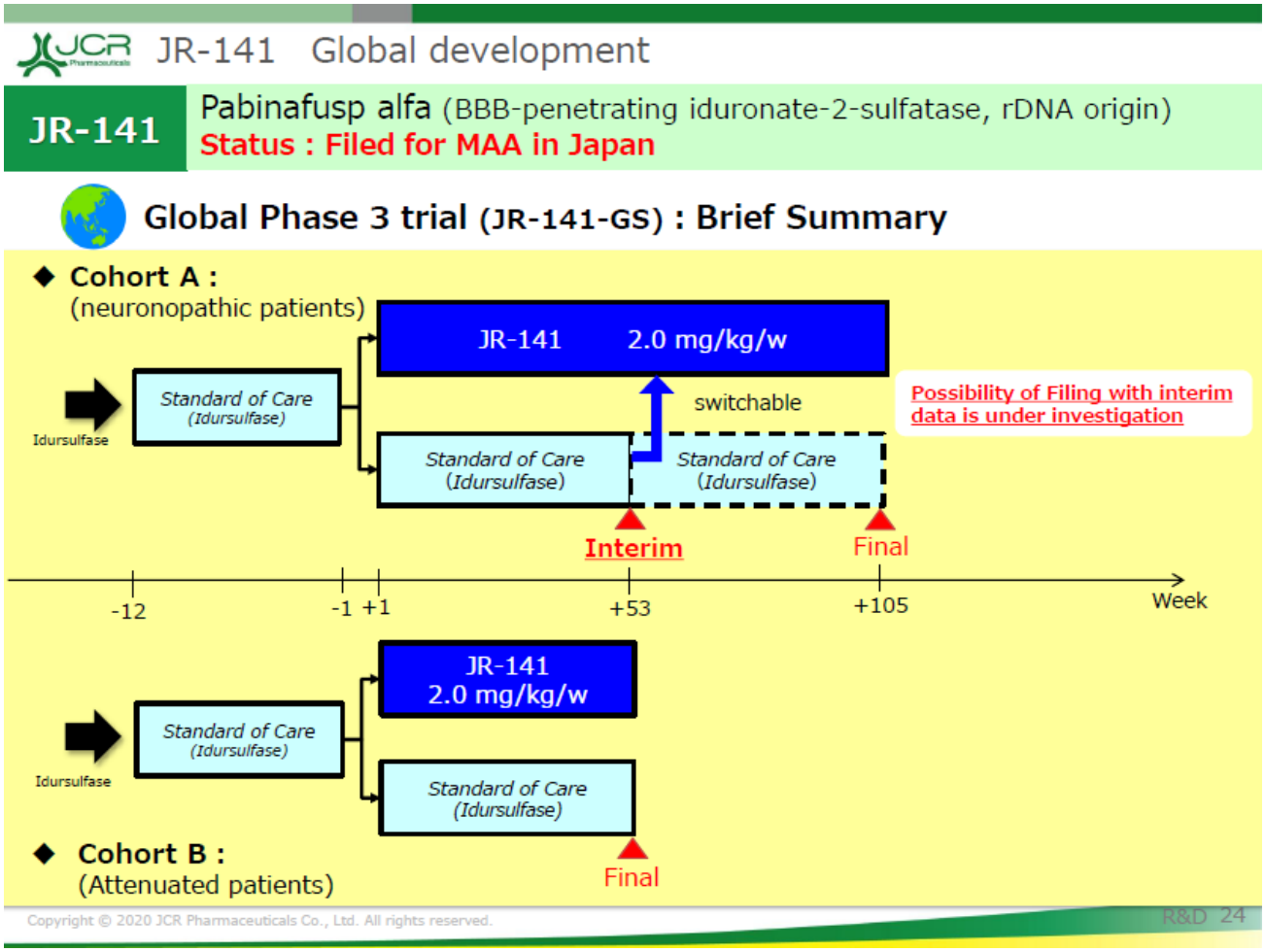


From this slide, I would like to describe the global Phase 3 clinical trial currently planned.

The three areas where this is scheduled to be implemented are the U.S., Brazil, and Europe. The objective of the study is to show the efficacy of the treatment against the central nervous system and systemic symptoms.

One of the characteristics of the trial is that we have set up two groups of patients. Also, the control group will be composed of patients receiving the standard treatment. We plan to recruit approximately 50 patients for this trial.

The primary endpoints are CSF heparan sulfate concentration, CNS symptoms, and systemic symptoms.

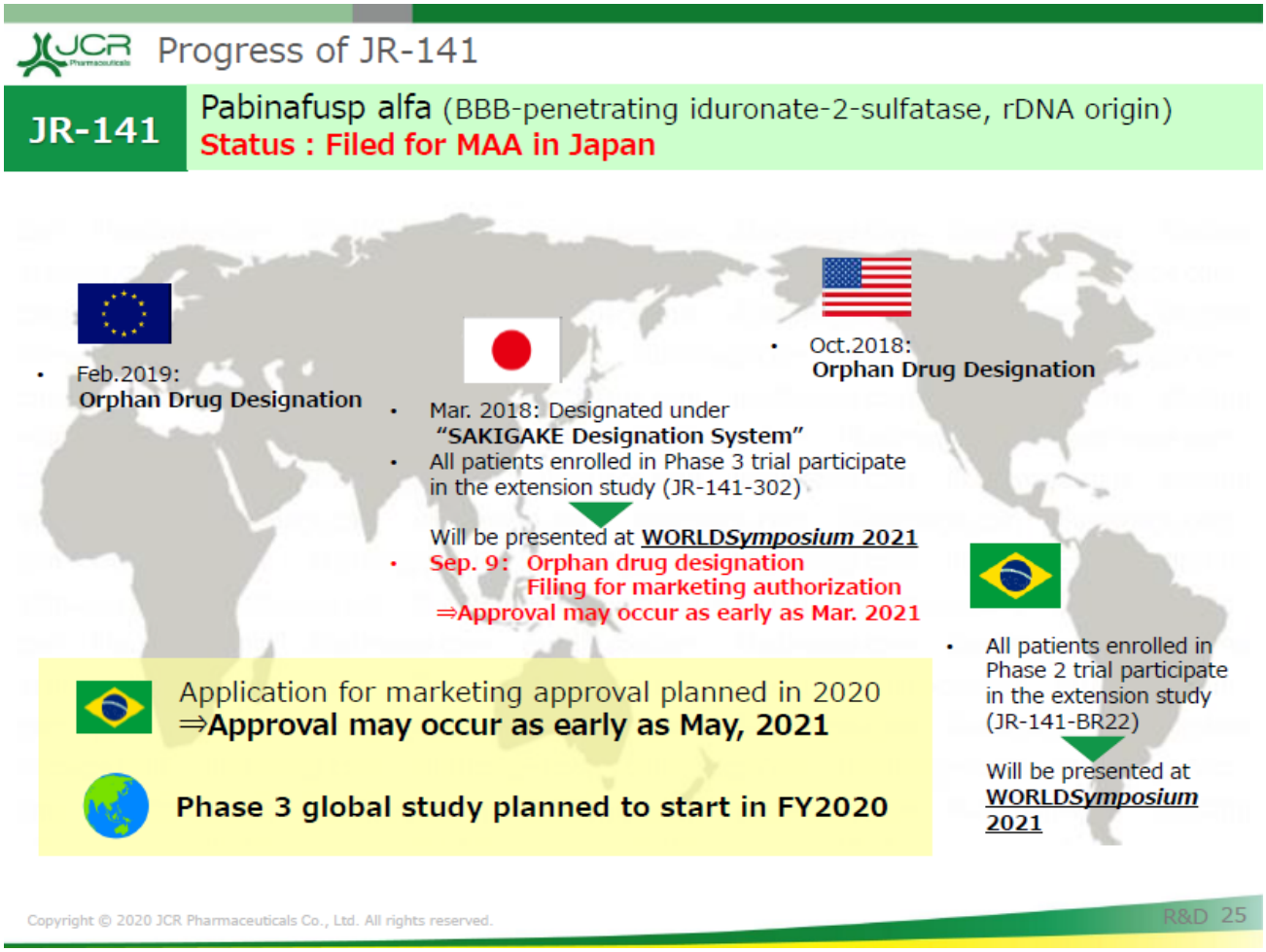


This is an outline of the trial.

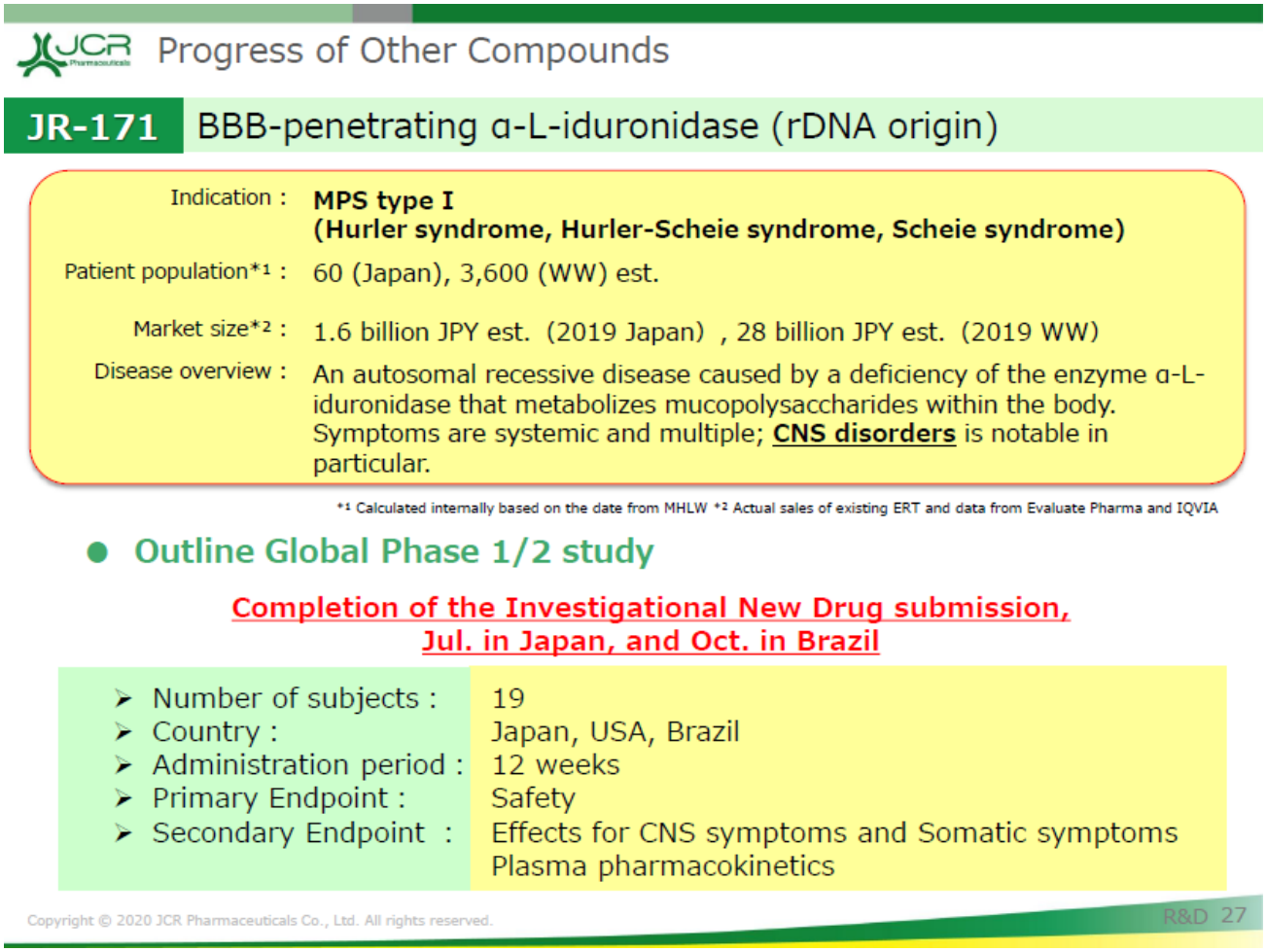
For Cohort A, we will be recruiting subjects aged three to six years of age with severe type. Since this disease is characterized by a gradual deterioration of the central nervous system symptoms, we have set a two-year assessment period for Cohort A.

However, many experts have expressed their ethical concerns regarding the continuation of standard treatment for two years, which is not expected to be effective for central nervous system disease. To resolve this issue, we have set the following points in this study: after 52 weeks, if certain criteria are met, the group will be switched to receive JR-141.

Cohort B is an analysis for one year. Therefore, as you can see, based on the results of the one-year analysis in Cohort B, and the results of the interim analysis in Cohort A, it may be possible to submit an application based on interim data.

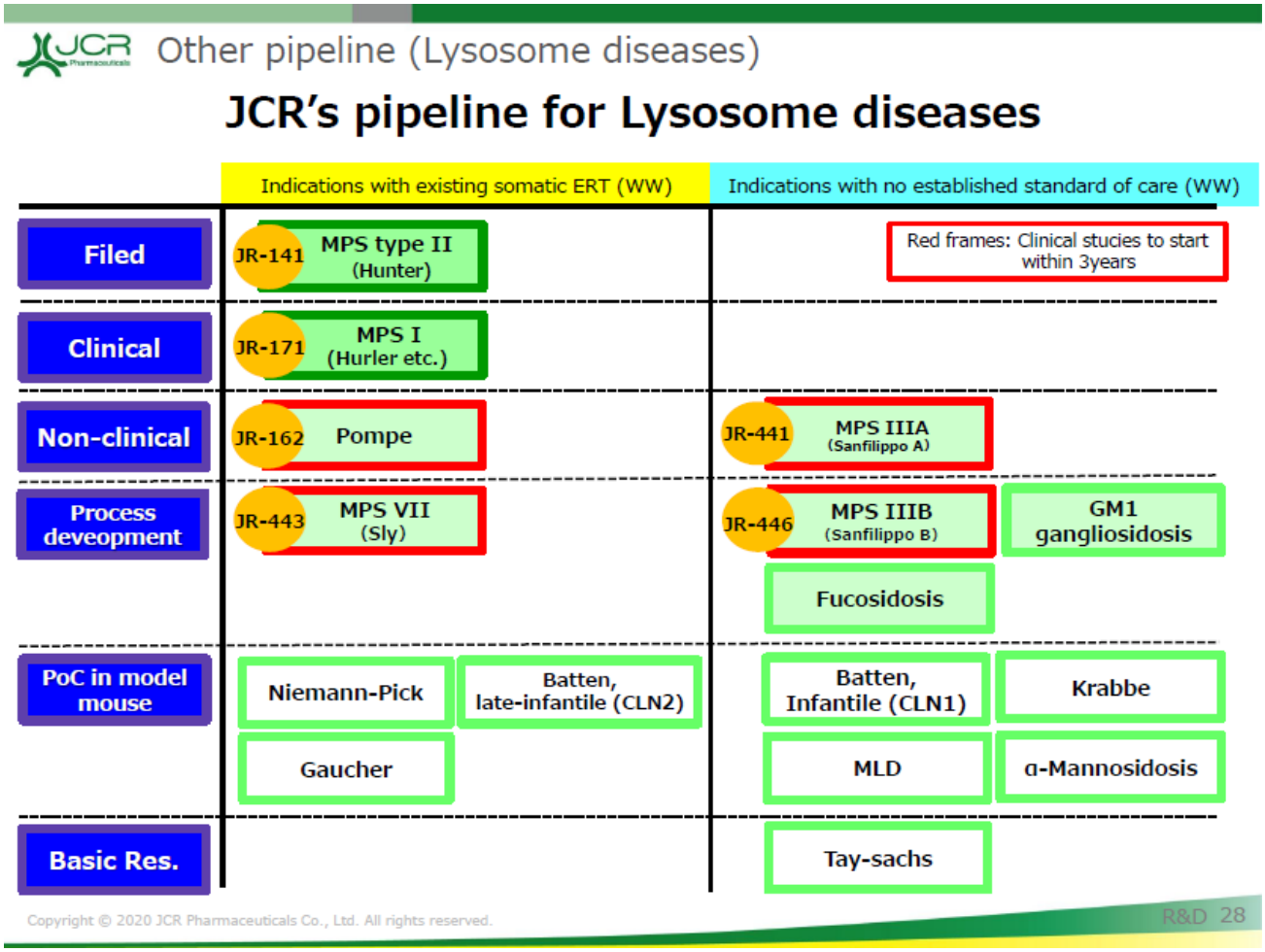


I mentioned the status of the application in Japan earlier. In Brazil, we plan to apply for marketing approval in 2020. If the application proceeds as expected, it is expected that approval will be obtained in May 2021.



This slide is an explanation of JR-171, which we are currently developing.

JR-171 is a treatment for type I mucopolysaccharidosis. There are currently no effective treatments for the central nervous system effects of this disease, so there is a clear unmet medical need. We have submitted trial notices in July 2020 in Japan, and in October in Brazil. We intend to proceed with recruitment in the near future.



This shows the stages of drugs that JCR is developing for lysosomal disease.

We plan to develop JR-441 for mucopolysaccharidosis type IIIA as shown on the right. Within the next three years, we also aim to conduct clinical trials of JR-162 for Pompe disease, JR-443 for mucopolysaccharidosis type VII, and JR-446 for mucopolysaccharidosis type IIIB.

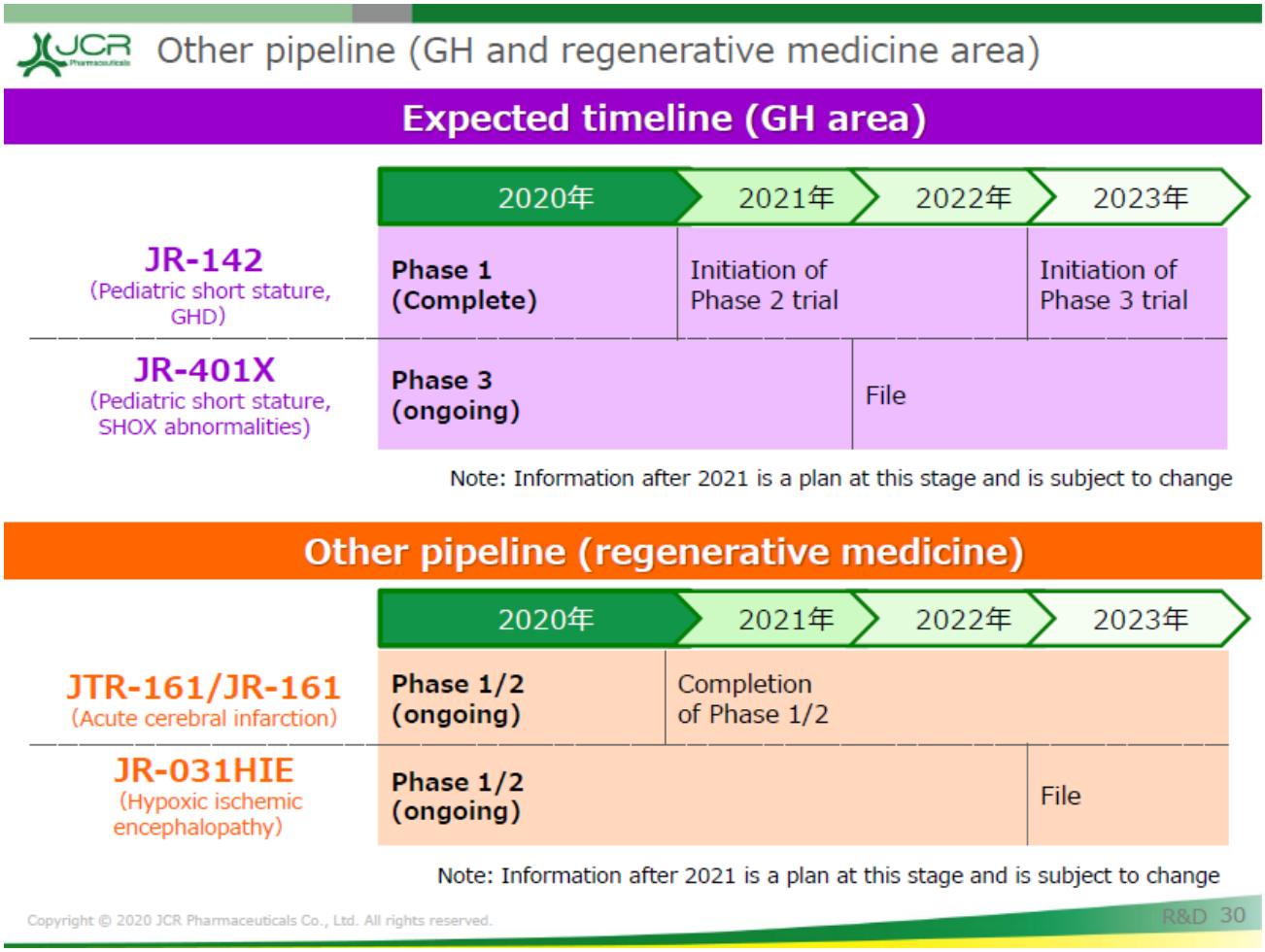
Other pipeline (Lysosome diseases)

Expected timeline (Lysosome diseases)

	2020	2021	2022	2023
JR-141 Pabinafusp alfa (MPS II)	**Japan : Filed** Brazil : To be filed	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 2021 is a plan at this stage and is subject to change

The timeline is shown here. As I mentioned earlier, with regard to JR-441, we are aiming to commence trials in 2022.



Clinical trials are also underway in the growth hormone field. In 2020, we completed Phase 1 clinical trials for JR-142 and long-acting growth-hormone formulations. We are currently in discussions with the authorities and specialists in preparation for the start of Phase 2 trials next year.

This is a project for expanding the indications for GROWJECT®, JR-401X, SHOX deficiency disorders. We have completed registration in June of this year too, so we would like to apply as planned as soon as we get the data. Regarding regenerative medicine development, JTR-161 is being developed jointly with Teijin Limited. In addition, we are progressing well in the final stage of recruitment with a trial for JR-031HIE, a drug developed in-house for treatment of neonatal hypoxic-ischemic encephalopathy.

This concludes my presentation on the progress of drug development in the areas of lysosomal disease, protein preparations, and regenerative medicine. Thank you very much.

[END]

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