JCR Pharmaceuticals Co., Ltd.

Givinostat Update

July 15, 2026

Event Summary

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[Participants]	
[Number of Speakers]	4

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Presentation

Moderator: We will now begin the Givinostat Update of JCR Pharmaceuticals Co., Ltd.

I would like to make a note of this before the briefing begins. The transcript and video of today's presentation and question-and-answer session will be posted on our official website at a later date.

Please also note that today's presentation may include forward-looking statements based on current expectations, and that all such statements are subject to risks and uncertainties. Today's presentation and the materials used are intended to provide information to shareholders, investors, and members of the media, and are not intended for promotional or advertising purposes, nor do they constitute medical advice.

I would like to introduce today's attendees.

Toru Ashida, Representative Director and Chairman.

Ashida: This is Ashida.

Moderator: Hiroyuki Sonoda, Representative Director and President.

Sonoda: This is Sonoda. Thank you.

Moderator: Yoh Ito, Managing Executive Officer and Executive Director of the Corporate Strategy Division.

Ito: This is Ito. Thank you.

Moderator: Kazunori Tanizawa, Expert Fellow and Director of the New Product Planning Office, Corporate Strategy Division.

Tanizawa: This is Tanizawa. Thank you.

Moderator: We have four attendees.

The materials for this briefing have been posted on our website today. If you need the materials, please find them there.

The presentation, including the question-and-answer session, is scheduled for about one hour. The question-and-answer session is scheduled for approximately 40 minutes, and we will take all questions following the presentation.

First, I'd like to begin with a few words from Ashida. Thank you.

Ashida: Thank you very much for taking time out of your busy schedule to attend our briefing today. I am Toru Ashida, the Chairman.

As we announced earlier this morning, we have decided on a domestic development plan for givinostat, a treatment for Duchenne muscular dystrophy that we acquired from Italfarmaco. Based on consultations with the PMDA, we have been conducting a series of discussions with Italfarmaco, the Licensor.

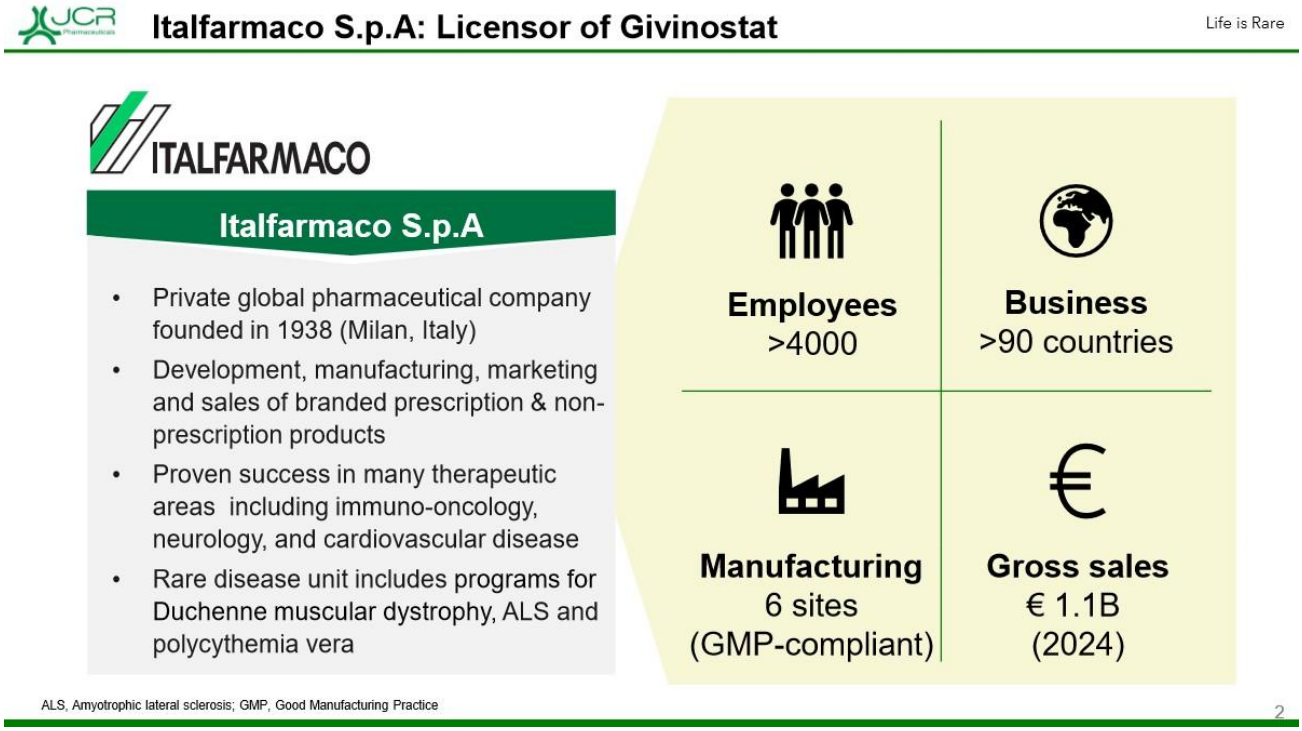
As a result, we will move up the timeline we previously announced and aim to submit the application by the end of this year, with the goal of obtaining approval and launching sales by the end of 2027.

Givinostat has already been approved by the FDA and the EMA. We believe that the launch of this drug will help eliminate drug loss and shorten the drug lag in Japan, thereby contributing to the fulfillment of patients' unmet medical needs.

Furthermore, from the perspective of our company's revenue structure, this is a highly promising and significant drug product with the potential to significantly transform our future sales and profit.

Next, President Sonoda will explain the details of the development plan.

Thank you for your time today, and we appreciate your attention through to the end.



Sonoda: This is Sonoda. Thank you for joining us today.

Now, I'd like to begin by explaining the givinostat update.

First, I'd like to say a few words about Italfarmaco, the licensor of givinostat.

As described here, it is a privately held global pharmaceutical company based in Milan, Italy that was founded in 1938.

The company operates in more than 90 countries, where they are engaged in the development, manufacturing, marketing, and sale of pharmaceuticals.

They develop and market drugs in a variety of fields, one of which is rare diseases. This means that Duchenne muscular dystrophy and this drug, givinostat, are included among them.

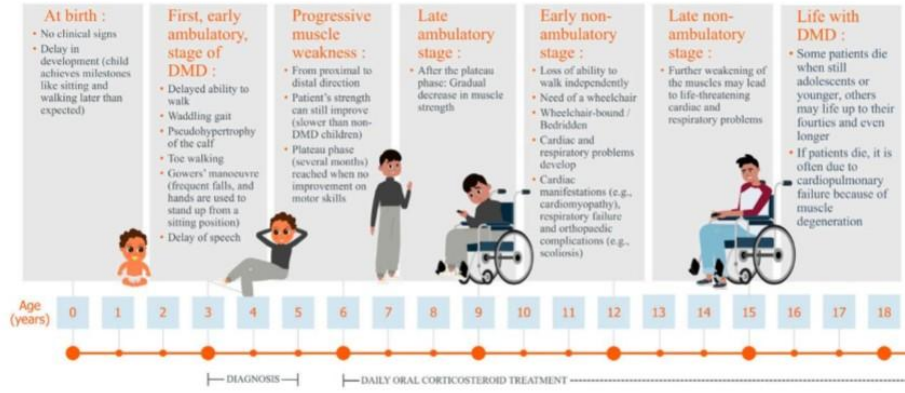
Overview: Duchenne Muscular Dystrophy (DMD)

Life is Rare

【Etiology・Clinical course[1]】

- Caused by mutations in the dystrophin gene, leading to the absence of dystrophin protein
- Onset at 3–5 years of age, progressive motor decline; loss of ambulation around 10 years

【Life cycle of the patients with DMD[2]】



DMD, duchenne muscular dystrophy

1. Clinical practice guidelines for Duchenne Muscular Dystrophy 2014 (Japanese) 2. Desmet T, et al., *Front Pharmacol*. 2025; 16: 1662586.

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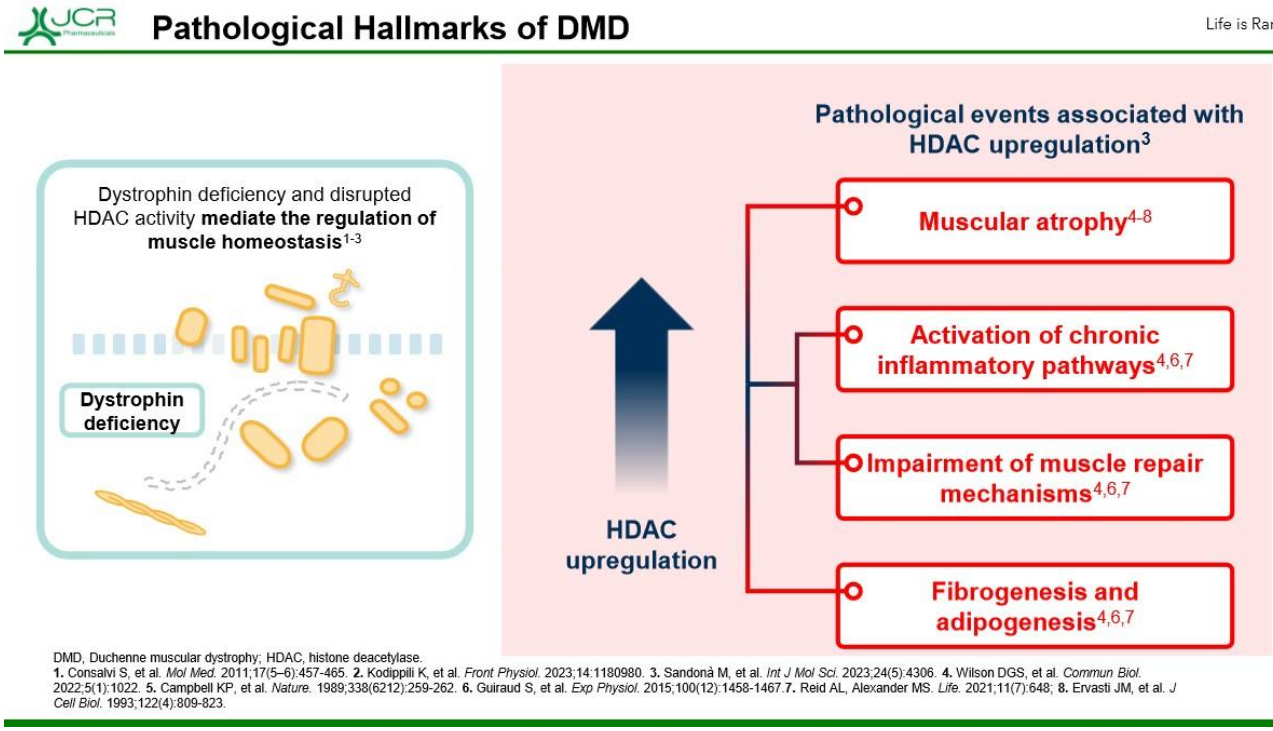
This drug, givinostat, is used to treat Duchenne muscular dystrophy (DMD). This slide explains what DMD is.

DMD is caused by a deficiency of the dystrophin protein. Therefore, I believe it can be classified as one of the so-called genetic diseases in which the dystrophin gene is mutated or missing.

Symptoms typically appear between the ages of three and five. Motor function begins to be impaired around age five, and it is said that many patients lose the ability to walk around age 10.

Since this is a genetic disorder caused by the absence of a gene present from birth, symptoms gradually progress from birth, eventually leading to an inability to walk.



This slide illustrates the pathological characteristics of DMD.

As I mentioned earlier, the body cannot produce a protein called dystrophin. When this is lacked, a series of cascading events follows, and as a result, the activity of histone deacetylase—abbreviated as HDAC —increases.

HDAC is originally regulated within the muscles. If this is not controlled, the expression of various genes will not proceed normally, so this is a very critical issue. In other words, the absence of dystrophin weakens the muscle structure, and the absence of dystrophin also increases the HDAC activity, which in turn leads to the emergence of various symptoms.

This drug does not directly support dystrophin itself. Rather, it is a medication that mitigates the other effects caused by the loss of dystrophin.

The pathological events that will take place are listed on the right Muscular atrophy, chronic inflammation, impairment of muscle repair mechanisms, and fibrogenesis and adipogenesis of muscles may occur. This means that such events occur as a result of increased HDAC activity.

Givinostat is a potent pan-HDAC inhibitor

Inhibition of the upregulated HDAC in DMD **redresses the imbalance in muscle homeostasis**, leading to[1,2]:

- **Increased** muscle repair and regeneration mechanisms[3,4]
- **Reduced** fibrosis and fat deposition[3]
- **Delayed** disease progression with preservation of functional capabilities[5,6]

DMD, Duchenne muscular dystrophy; HDAC, histone deacetylase.
1. Consalvi S et al. *Mol Med.* 2011;17(5-6):457-465. **2.** Kodippili K, Rudnicki MA. *Front Physiol.* 2023;14:1180980. **3.** Bettica P et al. *Neuromuscul Disord.* 2016;26(10):643-649. **4.** Sandonà M et al. *EMBO Rep.* 2020;21(9):e50863.
5. Mercuri E et al. *Lancet Neurol.* 2024;23(4):393-403. **6.** ClinicalTrials.gov. NCT02851797. Updated 2 February 2023. Available at https://clinicaltrials.gov/study/NCT02851797. Accessed August 1, 2024. 5

Givinostat is a drug that helps suppress this phenomenon.

The structural formula of the molecule is shown on the left.

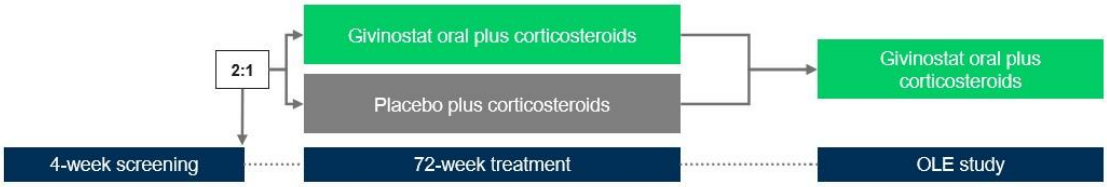
Earlier, I mentioned that elevated HDAC levels can be problematic. Givinostat suppresses that increase. In other words, it inhibits HDAC, so it's called an HDAC inhibitor.

To reverse what I just said, this suppresses that increase. So, it promotes muscle repair and regeneration, inhibits muscle fibrosis and fat accumulation in the muscles, as shown on the right side, and, as a result, slows the progression of the disease and helps maintain motor functions, such as walking.

EPIDYS: Phase 3 Study Design[1-3]

Life is Rare

- Randomized, double-blind, parallel-group, placebo-controlled study with a total of 179 ambulant boys randomized 2:1 (givinostat:placebo)
- Givinostat or placebo were administered in addition to corticosteroids



OLE, open-label extension.
1. Mercuri E et al. *Lancet Neurol*. 2024;23(4):393-403. **2**. ClinicalTrials.gov. NCT02851797. Updated February 2, 2023. Accessed May 9, 2024. https://clinicaltrials.gov/study/NCT02851797
3. Vandenborne K. Oral presentation at Muscular Dystrophy Association Clinical & Scientific Conference; March 19-22, 2023; Dallas, TX, USA.

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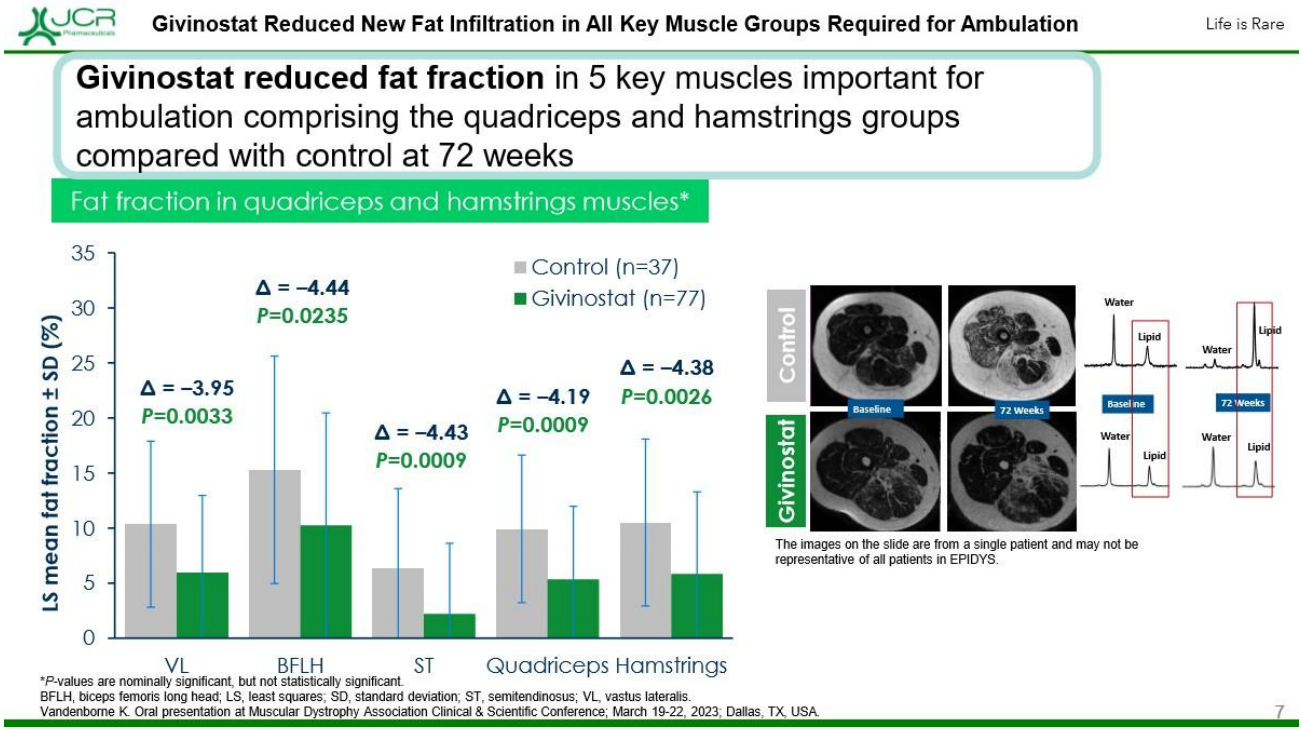
This clinical trial demonstrated the efficacy of this drug.

What we are presenting here is a Phase III clinical study conducted overseas by Italfarmaco. The design is shown here.

They are recruiting 179 patients for a randomized, double-blind trial. It is comparing the placebo group with the active treatment group.

This is a study in which comparisons are made over a 72-week treatment period, and the results are being evaluated.



Here are the results.

What is shown here is the process of fatty infiltration. This data shows that fatty infiltration in muscle tissue was suppressed.

On the right side are images. Here, the control, shown in gray, represents the placebo group, while the green one represents the group that received givinostat. They're measuring the extent to which muscle is being converted into fat over a period of 72 weeks.

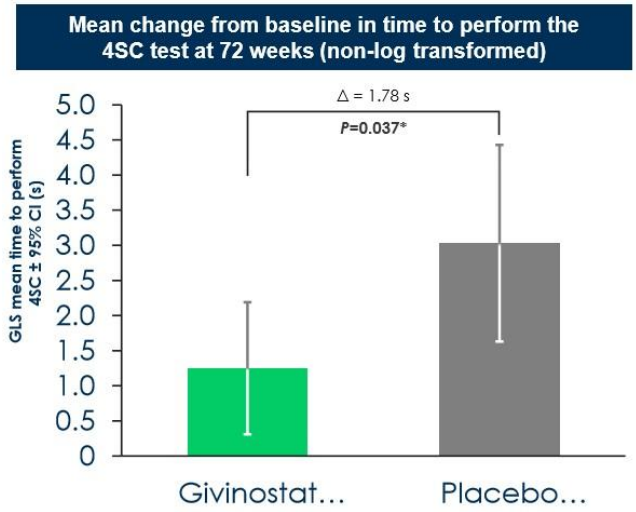
The bar chart on the left shows the results of that plot. Here, too, when comparing gray and green, the higher the gray bar, the more advanced the fatty infiltration is. Therefore, a lower bar is better.

The placebo is gray, the active drug, givinostat, is green. As you can see here, the group that was administered givinostat, shown in green, shows less progression of adipogenesis, and it has been suppressed. This means that muscle mass is being maintained.

EPIDYS Study: Primary Endpoint — 4SC at Week 72[1]

Life is Rare

- At week 72, givinostat plus corticosteroids reduced the decline in time to perform the 4SC test by 1.78 s when compared with placebo plus corticosteroids



*Data are means and 95% confidence intervals. The confidence intervals have not been adjusted for multiplicity and should not be used for hypothesis testing. Baseline mean values were 3.39 s and 3.48 s for the givinostat and placebo groups, respectively. All patients were also receiving systemic corticosteroids in a dose and regimen that was to remain unchanged over the follow-up period.
4SC, 4-stair climb; GLS, geometric least squares; s, seconds.
1. Mercuri E et al. *Lancet Neurol*. 2024;23(4):393-403.

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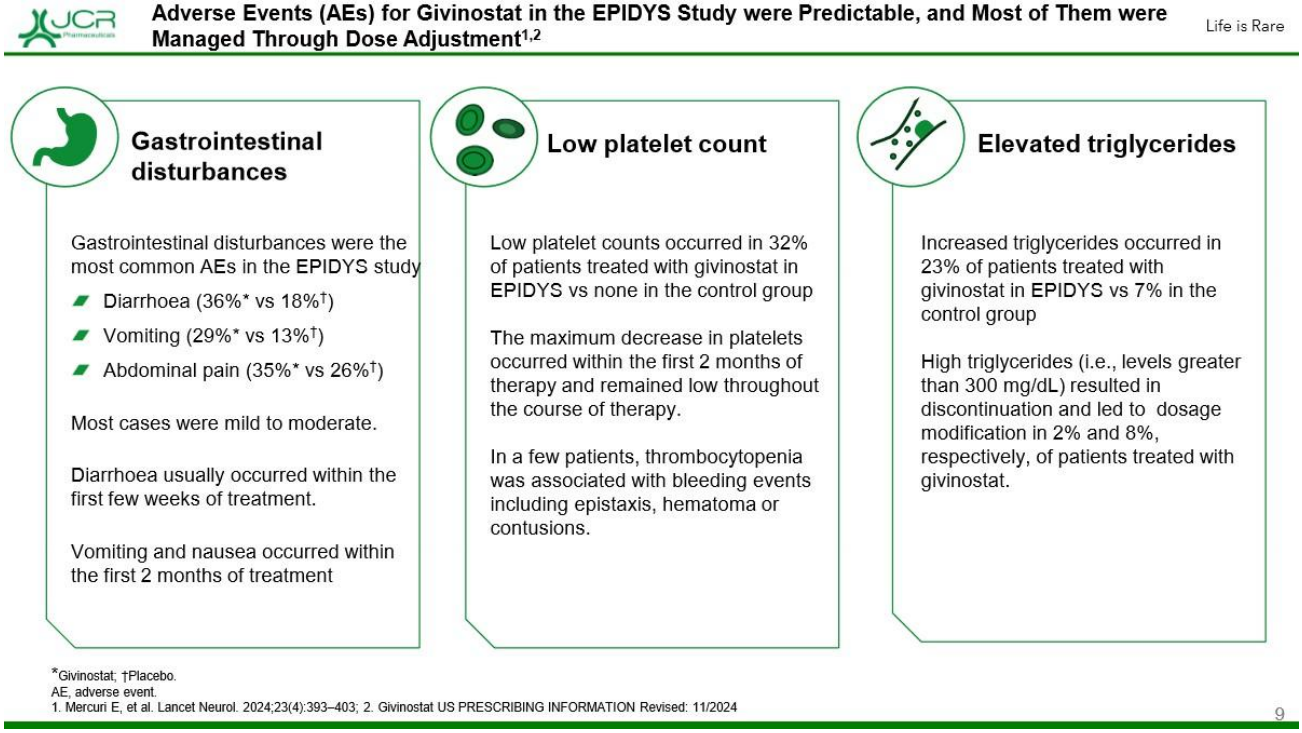
Now, the question is how that relates to the clinical symptoms .

This has been designated as the primary endpoint. It's called the 4SC test, which measures the time it takes to climb a four-step staircase.

These measurements were taken after 72 weeks of administration. Givinostat is shown in green and the placebo in gray. Since these measures the speed to climb, it's better to climb faster. In other words, the smaller the bar, the more effective it is.

In this case, it does not mean that the rate of climb speed will increase. As I mentioned earlier, since this is a progressive disease, we look at it as an indicator of how well we can slow down its progression.

In other words, compared to the placebo group that did not receive the medication, after 72 weeks, the green group that received givinostat showed a change from baseline that was 1.78 seconds shorter. This means that the time it took to climb did not increase to that extent.

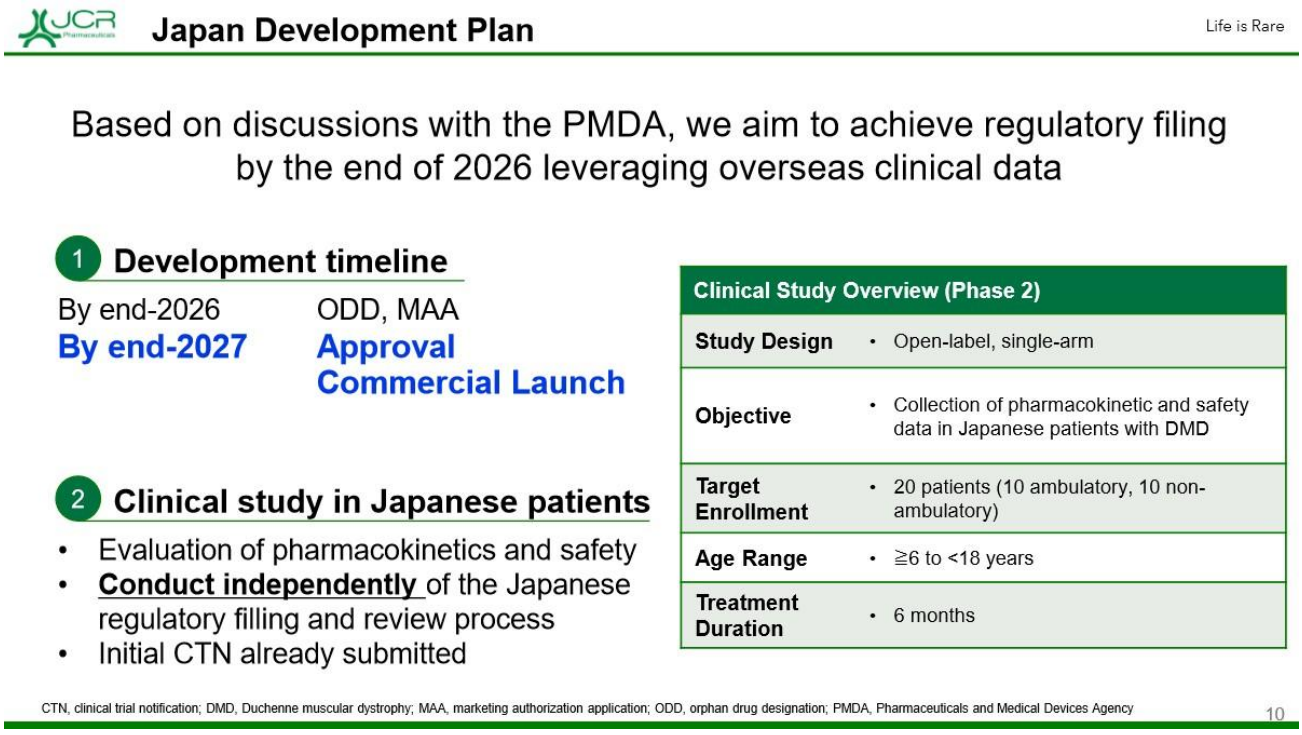


Next, let's discuss adverse events.

This drug is a low-molecular-weight compound, so adverse events have been reported.

As described here, adverse events, including gastrointestinal disturbances, were observed in approximately 20% to 30% of patients.

However, the reports indicate that most of these events were manageable and did not prevent continued administration of the drug.



I think this is where today's main topic begins.

Based on that data, we acquired the rights to this drug for the Japanese market last December and have been formulating a development plan.

After consulting with the PMDA and exchanging various communications and ultimately based on the results of the consultation meeting, we plan to submit an new drug application this year, 2026, based on the overseas data, specifically the clinical trial data from overseas that I mentioned earlier.

Item one on the left, development timeline. The same things were described here. We aim for marketing authorization application (MAA) by the end of 2026, obtain approval by the end of 2027, and launch.

In addition, we will conduct a clinical study targeting Japanese participants in parallel with MAA—this is the second point. It will be conducted independently of the MAA and review process I mentioned earlier. In other words, while we will submit the MAA based on data from overseas, data on Japanese subjects will be collected separately through clinical trials conducted in Japan.

The right side shows an overview of the clinical study. It’s the green table on the right. The study design is an open-label, single-arm study. We plan to recruit a total of 20 patients, 10 ambulatory and 10 non-ambulatory, for a six-month treatment duration.

Unmet Medical Needs in DMD Treatment

Life is Rare

Only two therapies approved in Japan (excluding steroid therapy)

	Approval status			Target DMD Patients[1]
	Japan	US	EU	
Gene therapy	✓2	✓	—	Japan; Negative for anti-AAVrh74 antibodies Ambulatory patients 3 years to less than 8 years
Exon skipping	✓2	✓	—	Japan; Patients with specific genetic mutations[3]

Eligible patients remain limited by age and genetic mutation criteria, leaving substantial unmet medical needs in DMD

Givinostat	—	✓	✓2	**US;** **≥6 years** **EU;** **Ambulatory patients ≥6 years on concomitant steroid therapy**

AAV, adeno associated virus; DMD, Duchenne muscular dystrophy
1. Based on labels for each product 2.Conditional approval 3. Patients who have a confirmed mutation of the DMD gene that is amenable to exon 53 skipping

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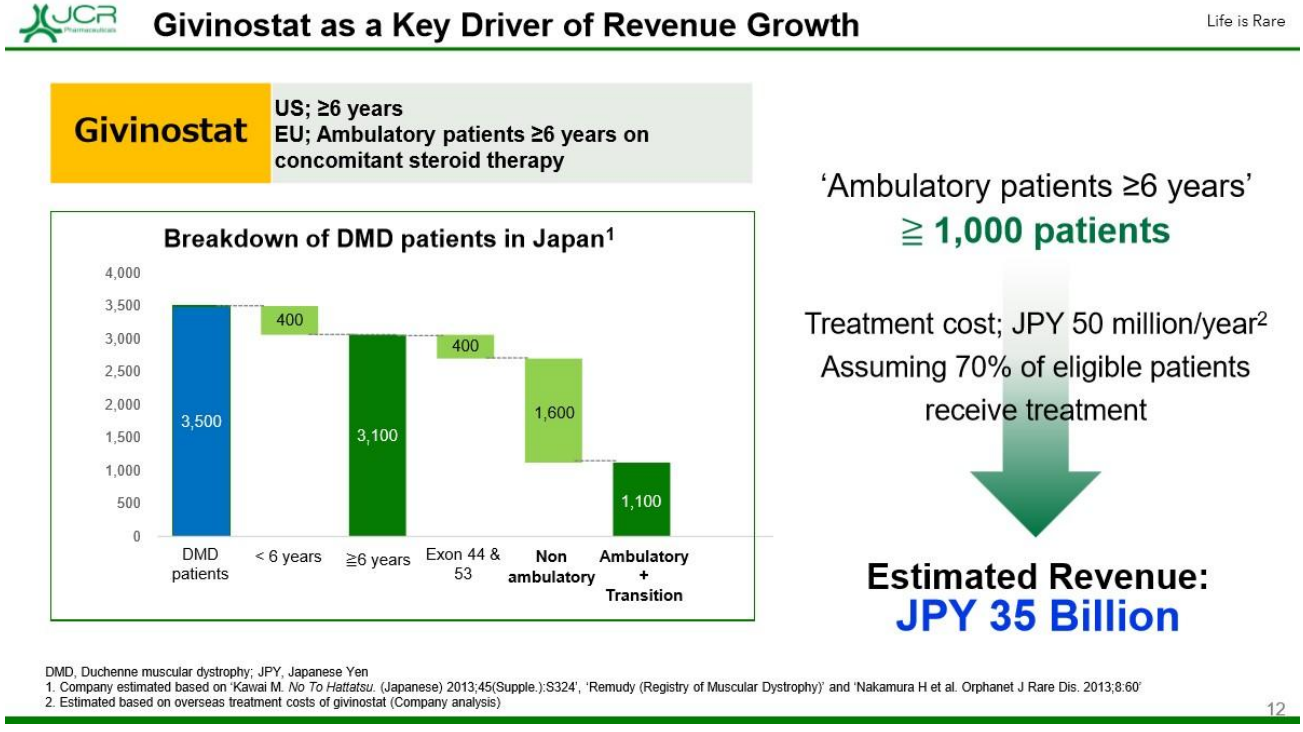
Next, I'd like to discuss the unmet needs in DMD treatment and how givinostat addresses those unmet needs.

Currently, two drugs other than steroids are approved in Japan. One is gene therapy, and the other is exon skipping.

However, these existing drugs have limitations, such as restrictions on the age range for administration, a narrow therapeutic window, and the fact that they are only effective in patients with specific genetic mutations, which result in a very limited pool of eligible patients and represent an unmet medical need.

In contrast, givinostat, which is approved for patients aged six and older in the US and Europe, can be administered to patients aged six and older regardless of genetic mutation.

We believe that givinostat will now be able to reach a broader range of patients than the two drugs mentioned above, including DMD patients who were previously unable to receive treatment even after those drugs were approved.



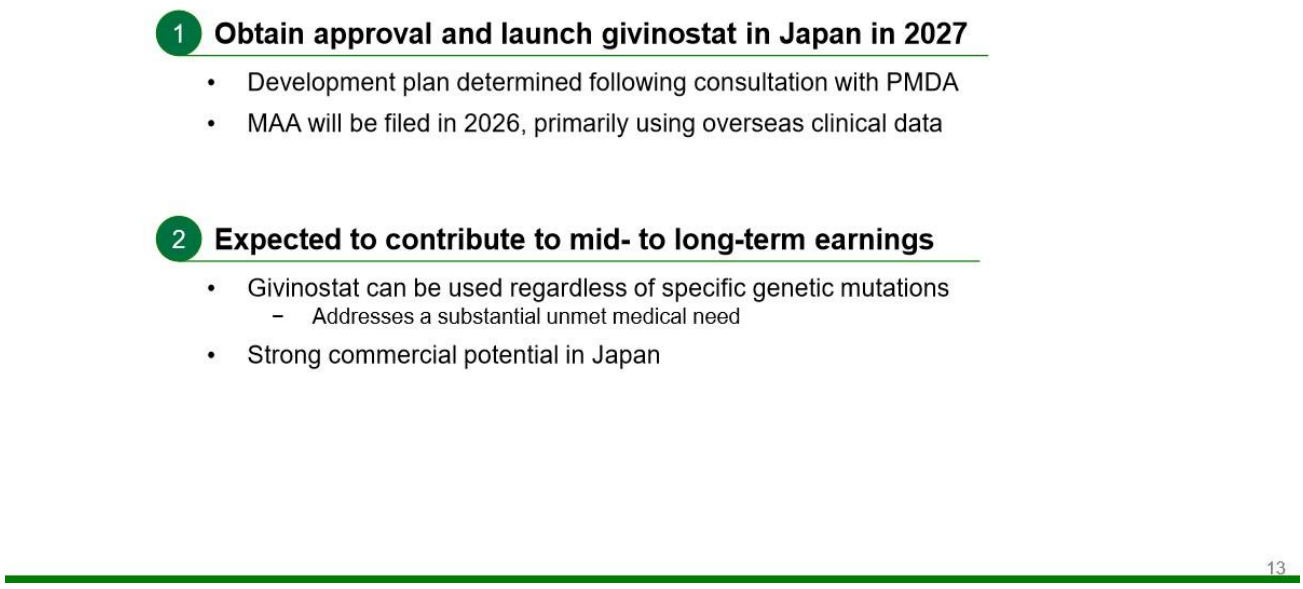
Next, let's talk about the business side.

Based on what I just mentioned, givinostat has the potential to be administered to patients aged six and older, regardless of genetic mutations.

The green box on the left shows how many DMD patients in Japan met those criteria. Of all DMD patients, represented by the blue bar, we estimate that the green bar on the far right represents approximately 1,100 ambulatory patients .

Moving on to the right, there are ambulatory patients aged six and older. I mentioned earlier that there are 1,100 of them, but I believe there are probably more than 1,000 in Japan.

Assuming that 70% of those patients use this drug and that the annual treatment cost is JPY50 million, the calculation shows that sales would amount to approximately JPY35 billion. Given the scale of our business, this figure would have a significant impact.



This is the last slide for today. Today's summary.

Number one, we aim to obtain approval and launch by the end of 2027. As I mentioned earlier, based on the results of the consultation with the PMDA, we have reviewed our development plan and MAA timeline, and this is our current thinking.

Number two, we believe this will make a significant contribution to our profit over the medium to long term. The reasons are as I have mentioned earlier. Givinostat is not dependent on genetic mutations, which means it can be administered to a very wide range of patients. Therefore, the figure of JPY35 billion I mentioned earlier represents a significant impact and potential given the scale of our business.

That's all from me.

Question & Answer

Moderator [M]: Now, we move on to the question-and-answer session.

We will first take questions from analysts, followed by questions from the media. Please note that questions will be answered in a one-question-at-a-time format, with a limit of two questions per person per session. However, you may raise your hand as many times as you like.

Now, we begin the question-and-answer session. First of all, Mr. Muraoka, please ask your questions.

Muraoka [Q]: Thank you very much. This is Muraoka from Morgan Stanley MUFG Securities.

Since the product is expected to receive approval and be launched next year, I think it will have a significant impact on next year's financial results. As this is a material issue, I'd really appreciate it if you could provide more details.

How should we compare the scale of the milestone payment upon regulatory approval to the JPY1.5 billion paid during the initial launch? And how will this affect P&L, and how should we be thinking about it at this point? I believe we've discussed this several times before, but I'd like to ask you again.

Ito [A]: I'll answer that for you. Thank you for the question.

As I mentioned before, the amount is not disclosed. I imagine we'll be giving a similar explanation when we present next year's budget. Assuming a milestone payment was made upon approval, that payment can be capitalized and amortized. Therefore, I believe the expense will be recognized on a pro-rata basis over a certain number of years.

Muraoka [Q]: Should I consider approval and launch separately?

Ito [A]: No, rather than treating it as a separate matter, I recall it as having been a single milestone at that time.

Muraoka [Q]: Understood. Thank you. So, the information that can be disclosed hasn't changed much from a few months ago, right?

Ito [A]: That's true regarding that point.

Muraoka [Q]: Understood. Thank you. Now, on to the second question. I'd appreciate it if you can disclose more compared to before.

I'd like you to elaborate a bit more on how the product is actually being used in the US after its launch. For example, things like what kind of patient profiles this is used for, in what situations it isn't used, how long it's typically used for, and which other medications it's most commonly used in combination with. It doesn't matter what specifically, but any information you could share would be greatly appreciated.

Tanizawa [A]: Thank you for your question. This is Tanizawa.

As for the US, we do not have detailed information on sales or other figures. On the other hand, the package insert includes information on both ambulatory and non-ambulatory. Another difference in the approval status compared to Europe is that there are no restrictions on the use of steroids.

In that sense, based on the information available, I have, of course, heard that this medication is being used even for non-ambulatory patients. I've also heard that patients are not on steroids are using it as well.

As for the last question regarding combined use, I understand that some patients are using it in various combinations. That is the information we currently have.

I believe it was approved in 2024, so now that two years have passed, that's how it's currently being used.

Muraoka [Q]: Thank you very much. Could you provide a little more detail on that, for example, perhaps with some specific figures, such as what percentage of patients fall into this category? Is there any concrete information like that you could share?

Tanizawa [A]: At this stage, we are not in a position to specify the exact number of patients.

Muraoka [M]: Understood. That is all from me. Thank you.

Moderator [M]: Thank you very much.

Next, Mr. Hashiguchi, please ask your questions.

Hashiguchi [Q]: This is Hashiguchi from Daiwa Securities. Thank you for taking my questions.

I suppose it will ultimately depend on the results of the reviews. I would like to hear your company's current assessment of what the indications and the approval process are likely to look like.

As you just mentioned, the criteria for the concurrent use of steroids, as well as the distinction between ambulatory and non-ambulatory patients, differ between the US and Europe. I think it's conditional approval in Europe, while it's standard approval in the US. I'd appreciate it if you could share your thoughts on how this might be handled in Japan.

Tanizawa [A]: Thank you for your question. This is Tanizawa.

To put it simply, my answer is that we are aiming to meet the conditions for approval in the US. The background to this is that we do, in fact, have frequent discussions with members of the DMD and muscular dystrophy communities.

As for the needs of both healthcare professionals and patients, there is a very strong desire, even among those who are non-ambulatory and use wheelchairs, to maintain their functional abilities as much as possible. From that perspective, we, including the authorities, have been discussing these matters.

One more thing, as for steroids, there are various opinions among doctors regarding this as well. I have heard that, if we are to consider the need for a variety of treatment options, it is naturally better to have no restrictions in that regard. Based on that need, we are currently engaged in ongoing discussions.

Hashiguchi [Q]: Thank you very much. Nevertheless, given the explanation on page 12 regarding the JPY35 billion figure, why did you base your explanation on patients like 1,100 or 1,000? Based on what you just said, I think it might have been better to use 2,700 as the denominator. What do you think?

Ito [A]: Thank you, Mr. Hashiguchi. This is Ito.

You are quite right about that. However, we are presenting this figure specifically for ambulatory patients. As you mentioned, we are exploring the possibility of expanding the application to non-ambulatory patients, and we would appreciate it if you could view this as a significant upside.

Hashiguchi [Q]: I understand that you aim to make this product usable even by non-ambulatory patients, but should I assume that the range of possible use cases is still somewhat broad?

Ito [A]: I think that's a fair way to understand that point.

Hashiguchi [M]: Understood. Thank you. That is all from me.

Moderator [M]: Thank you very much. Does anyone have other questions?

Mr. Kawamura, please ask your questions.

Kawamura [Q]: This is Kawamura from SBI SECURITIES. Thank you for your explanation.

I believe I have a very good understanding of the top-line assumptions and such, but could you please explain how it will contribute to profit? This is probably a low-molecular-weight compound, and I imagine the cost is quite low, but what profit margin should we expect your company to make on this? I realize it's difficult to answer at this stage, since the drug prices haven't been set yet, but if there are any benchmarks or guidelines, could you please share them with us?

Thank you.

Ito [A]: Thank you for your question. This is Ito.

As you say, since the drug prices haven't been set yet, it's very difficult for me to give you a specific figure. As we have stated previously, from the perspective of costs associated with typical implementation contracts for this type of product, we believe we have secured a very favorable agreement for this contract itself. In that sense, we expect this drug to contribute significantly not only to sales, but also, naturally, to profit.

Kawamura [Q]: Understood. Thank you.

This overlaps quite a bit with Mr. Hashiguchi's question from a little while ago, but I think that, based on your preliminary consultation with the PMDA this time, you probably have a pretty good idea of the requirements and framework for submitting the MAA.

We understand that the goal this time is to meet the conditions for approval in the US, but is it correct to assume that the MAA was submitted with this as a given to some extent and that the process will proceed to the review stage based on that or, in other words, that this became clear during the preliminary consultation? Should I consider that to be such a significant factor?

This is my last question.

Tanizawa [A]: Thank you. This is Tanizawa.

At this time, we are unable to provide details regarding our discussions with the PMDA. However, as I mentioned earlier, we believe we have adequately explained the high level of domestic demand and unmet needs, so I would like to consider this my response.

Kawamura [M]: Understood. Thank you. That is all from me. Thanks again.

Moderator [M]: Thank you very much.

Then, Mr. Yamakita, please ask your questions.

Yamakita [Q]: This is Yamakita from Jefferies. Thank you for your explanation.

Here's my first point. This suggests that additional clinical trials will be required, and I got the impression that the PMDA may still have concerns regarding safety. Once approval is granted, is there a possibility that the requirements for monitoring adverse effects will become stricter? For example, reports might be required on a temporary basis or something along those lines. Basically, I want to confirm whether doctors might hesitate to use it because the prescription process and subsequent procedures have become too complicated.

Tanizawa [A]: Thank you for your question. This is Tanizawa.

I believe there was a question earlier about whether additional clinical trials are necessary, but it has long been recognized that when a drug has already been approved overseas and an application is filed in Japan, obtaining data specific to the Japanese population is very important. We strongly agree on that point as well. Given the differences between ethnic groups and various other factors, we believe that experience with Japanese patients will provide a great deal of valuable information.

Given that perspective, we believe that conducting clinical studies is a very reasonable course of action. Please don't interpret this as meaning we are asked to conduct the clinical studies because there are some major concerns. Rather, please understand that clinical studies are required as part of the standard process.

I believe your other question concerns the complexity of post-approval monitoring. The fact that this has already been approved in Europe and the US is very reassuring news for us. Since methods for monitoring safety, dosage, and adverse effects have generally been established based on patients' actual use in clinical practice, we intend to make the most of that expertise, as we conduct post-marketing monitoring.

Yamakita [Q]: Understood. Thank you. Sorry, just one more thing.

I think the reduction in fatty infiltration is probably the most significant benefit. Hasn't this effect been confirmed in gene therapy or exon skipping? I'm sorry. Maybe I should just read that paper carefully, but could you please explain it to me?

Tanizawa [A]: Thank you for your question. This is Tanizawa.

I haven't reviewed all the data from other companies, so I hope you'll bear with me on that point. With regard to this data, it holds a very important position for givinostat—as a PD (pharmacodynamic effects), so to speak—because it demonstrates clinical efficacy that supports the mechanism of action, and because it is quantifiable and shows a clear difference, this makes it a very important point. As for other companies, I'm sorry, but I don't really have any recognition of seeing anything like that at this point.

In light of this, since there was a change in the actual function, specifically the time it took to climb a four-step staircase, I presented this data because I believe it is an excellent example of how the mode of action leads to PD, which in turn leads to clinical symptoms.

Yamakita [Q]: Understood. Thank you. I do think it's probably fine, since there's solid data on clinical endpoints. In that regard, how much importance do doctors in Japan place on this decrease in body fat percentage? Are you getting any feedback domestically?

Tanizawa [A]: Thank you for your question.

I think what the doctors place the greatest emphasis on is, after all, which specific clinical symptoms showed improvement. I think this is true for them to evaluate other companies' products as well, but I get the impression that their primary focus is on improving functionality.

For example, in that section, the time for a four-stair climb showed a significant difference compared to the placebo group, and as also shown in the appendix, the maintenance of walking was extended by about 2.9 years to 3 years compared to the natural history group. I think this kind of data is likely to be accepted by doctors as a clear benefit, in fact.

Another point is what the underlying mechanism of action is, what findings have been observed in non-clinical studies, and what supporting data is available from clinical studies, and it is, of course, extremely important that these findings support the claim. Regarding this drug, we are able to fully explain not only the mode of action, but also the intermediate steps involved. I think one of those is the suppression of fatty infitration.

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

Moderator [M]: Thank you very much.

Next, Mr. Mizuno of Tokio Marine Asset Management.

Mizuno [Q]: This is Mizuno from Tokio Marine Asset Management. Thank you for taking my questions.

I think that's a really good situation. Based on what your company said during the financial briefing, I understand that you are considering introducing drugs for rare diseases in this manner going forward, not limited to this particular drug. Although it hasn't been approved by the cabinet yet, I believe that introducing such drugs into the domestic market is one of the key strategies in the growth strategy outlined in the basic policy.

Therefore, I would like to confirm whether your company is considering applying this approach to other compounds in the future. Also, as a potential clue for ensuring reproducibility if that is the case, I would appreciate it if you could explain the circumstances leading to this partnership with Italfarmaco, specifically how this agreement came about.

Sonoda [A]: Thank you for your question. This is Sonoda.

First, regarding your initial question if we are considering introducing other products in the same way. As I've mentioned before, we are considering this, if it aligns with our scale and area of expertise.

However, as in this case, the drug's efficacy has already been proven in Europe and the US, and Japan is the only market where it hasn't been approved yet. Additionally, there aren't that many that actually fit our field. Please don't take this to mean that we already have the next step in mind. Rather, our stance is that if something aligns with our objectives and what we're currently working on, we'll adopt it. That's how I'd like you to understand it. We're not in the phase of looking around opportunities.

I think the second question would be: How did you find this drug? In this regard, we are in discussions with various pharmaceutical companies both in Japan and overseas to explore opportunities to license out our technology or assets rather than through implementation, like the one we just discussed, to various companies. We have been in ongoing communication with various companies.

During our discussions with Italfarmaco, we learned that they were developing this drug for DMD in regions outside of Japan. This led to a discussion about whether JCR could potentially be involved in the development of this drug in Japan, given the relationship between our two companies.

Mizuno [M]: Thank you. That is all from me.

Moderator [M]: Thank you very much. Does anyone have other questions?

Now, I'd like to open the floor for questions, not just from analysts, but also from the media. Does that sound good?

Mr. Muraoka, go ahead.

Muraoka [Q]: Sorry. Thank you. This is my second time.

Regarding the data on body fat percentage from page seven. It's not the data itself, but the sample size, 37 in the control group and 77 in the givinostat group, for a total of 110 people. As you can see on the previous page, there were 179 people who took the exam. I have the impression that few patients were included in this analysis. I do feel like there aren't many numbers in the control group, but the ratio remains at one to two, and it's pretty much the same.

Do you have any idea why there were patients who couldn't be analyzed, or if there's any background to this? At first, I thought it might be a safety issue with givinostat, but it doesn't seem to be that. I also think there have been dropouts in the control group. It would be helpful if you could explain that point.

Tanizawa [A]: Thank you for your question. This is Tanizawa.

I'm sorry, but I cannot answer questions at this time regarding the background of this study or the criteria used to select patients. I'll double-check that.

Muraoka [Q]: So, when your company looked at this data, you didn't really have the same question as I did?

Tanizawa [A]: Since the analysis includes MRI measurements, I suppose there are various possibilities, including these measurements couldn't be taken. Like, not all the data is available, for example. I'd like to look into this to see if there might be possibilities in that area.

Muraoka [Q]: In any case, it seems like givinostat, in particular, has been administered for a long time. Is that correct?

Tanizawa [A]: Well, the details of the clinical study have already been published in a paper, so we can verify how many patients of what types were enrolled and how many completed the trial. As far as I remember, there were no instances of significant drop-offs or safety issues.

Muraoka [M]: Understood. Thank you. That is all from me.

Moderator [M]: Thank you very much.

Next, Mr. Ishii, please ask your questions.

Ishii [Q]: This is Ishii from Iyaku Tsushinsha.

Since this medication is intended to slow the progression of the disease, are there any screening methods or similar approaches available to detect the disease early?

Tanizawa [A]: Thank you for your question. This is Tanizawa.

While efforts are being made overseas to detect this disease through newborn screening, there is currently no such program in Japan designed to diagnose it immediately after birth. I believe that is recognized as an issue.

On the other hand, since there are widely administered screenings, such as the 1.5-year-old checkup and the three-year-old checkup, there are many cases where DMD or muscular dystrophy is diagnosed at those times. It is said that, compared to other countries, diagnosis in Japan tends to occur relatively early.

Ishii [Q]: Got it.

Next, regarding the clinical trials in Japan. Is it unlikely that this would lead to racial disparities?

Tanizawa [A]: As for this, my answer is that we really won't know until we see the results. Depending on disease, there can be significant differences in the backgrounds of Japanese patients and patients from other countries. Based on what we have observed so far, there is no significant difference between Japan and other countries regarding Duchenne muscular dystrophy. Since I expect the disease progression follows a similar clinical course, I believe that possibility is unlikely.

Ishii [M]: Thank you.

Moderator [M]: Thank you very much.

Next Ms. Yamaji, please ask your questions.

Yamaji [Q]: Thank you. This is Yamaji from Nikkei Biotech.

First, I'd like to ask about the clinical trial you plan to conduct in Japan. The Phase III study conducted by Italfarmaco was carried out in combination with corticosteroids. Will the clinical study conducted in Japan also be conducted with the use of steroids?

Tanizawa [A]: Thank you for your question. This is Tanizawa.

As for the inclusion criteria and other requirements for the trial design, we have just submitted the clinical trial notification, and I would like to explain those details once it has been accepted and officially disclosed on the public information website.

Yamaji [Q]: Understood. Thank you. One more thing.

You have shown us Italfarmaco's Phase III data. While data supporting the value of givinostat indicated its ability to suppress fat infiltration in muscles, to what extent has the association between a reduction in fat percentage on MRI and the long-term maintenance of motor function been proven?

Tanizawa [A]: Thank you for your question. This is Tanizawa.

Rather than a direct correlation, it is generally understood that as DMD progresses, a process that naturally involves muscle atrophy, fibrosis, and fat infiltration, patients gradually lose the ability to perform certain tasks.

According to our givinostat data, for that specific aspect, such as climbing stairs, we also conducted the NSAA (North Star Ambulatory Assessment), and those results showed nominal significance, indicating functional

improvement. As a result, a detailed MRI examination of the findings confirmed that muscle mass had been maintained. I hope to be able to explain the concept of correlation in that context.

Yamaji [M]: Understood. Thank you.

Moderator [M]: Thank you very much.

Next, Mr. Okada, please go ahead.

Okada [Q]: This is Okada from Yakuji Nippo.

The first point is about how this product differentiates itself from existing drugs, so to speak. I think one of the key strengths of this drug is that it has a broad scope of application, as it doesn't depend on factors such as age or genetic mutations. If patients have the option to choose between their current medication and givinostat, could you please explain the advantages of givinostat, such as its benefits for patients, as well as whether the two can be used together?

Tanizawa [A]: Thank you for your question. This is Tanizawa.

The issue of concomitant use is a very important one. The first one is clinical superiority. Gene therapy, of course, is administered via injection, and exon skipping is also done via injection, whereas givinostat can be administered orally. I think that, in that regard, the method of administration is one of the factors patients consider when making their choice.

Another point is that, as you mentioned, the indication is fundamentally different, so to speak. I think the decision will be based on the fact that it can be widely used for patients aged six and older. Also, the eligibility of ambulatory or non-ambulatory patients for treatment also differs from drug to drug. so we need to take that into account.

As for concomitant use, the views expressed by academic societies and other such bodies after the product is released will also be very important in this regard. It is difficult for me to make any predictions at this point.

One fact, as I mentioned earlier, is that all three drugs have been approved in the US. We are aware that there are patients who are taking these medications in combination.

Going forward, regarding such combination therapies, for example, in the case of SMA, The Japanese Society of Child Neurology has issued Combination Therapy Guidelines. I think it's likely that these issues will be discussed among specialists in that context.

Okada [Q]: I imagine there's a difference between injections and oral medications when you have the option to choose between them, but how do they compare in terms of efficacy?

Tanizawa [A]: Regarding this point as well, I believe we will need to compare and contrast the efficacy and safety data for each formulation that the professors have published. While we will not comment on data from other companies, regarding givinostat, as I mentioned earlier, the fact that a statistically significant difference was observed in the primary endpoint in a placebo-controlled trial is, I believe, a very important point to highlight.

I believe the supporting data is also important when doctors explain this, and I think it's also important for patients to determine what kind of benefits they can expect.

Okada[Q]: Understood. Thank you. One more point.

Givinostat's market share overseas, you mentioned that sales were unable to disclose. What about market share? Europe and the US.

Tanizawa [A]: Earlier, I answered that it would be difficult to provide specific figures regarding the number of patients and other such details. I hope you'll understand market share from a similar perspective.

Okada [M]: Understood. Thank you very much.

Moderator [M]: Thank you very much.

Next, Ms. Innami, please ask your questions.

Innami [Q]: This is Innami from Toyo Keizai. Thank you for the explanation today. I have two questions.

This is my first question. You mentioned that you had initially expected to obtain approval by the end of 2028, but the timeline has been accelerated. Could you explain the reasons behind this acceleration? Specifically, were there any factors that emerged during your discussions with the PMDA that led to the timeline being brought forward sooner than your company had originally anticipated?

Tanizawa [A]: Thank you for your question. This is Tanizawa.

I don't think this is limited to the PMDA, but Japan is currently facing the challenges of drug lag and drug loss. I understand that there is an effort underway to address this issue on a national level.

We identified these issues based on our patients' needs, so we worked hard to get them approved as soon as possible, even if it was just one day sooner. We have presented our case to the authorities based on that rationale.

So, while I don't think there have been any major changes, what stood out in this case was the sense that everyone involved, including the stakeholders, was eager to obtain approval quickly and eliminate the delays and losses associated with drug lag.

Innami [Q]: Thank you.

My second question, this is also related to what I mentioned earlier about marketability. Theoretically, is this medication something you're supposed to keep taking for the rest of your life, even as you get much older, or is it the kind of medication that stops working at some point? I'd really appreciate it if you could give me a general thought of what it's like, even if the details are a bit complicated. Could you comment on this?

Tanizawa [A]: Thank you for your question. This is Tanizawa.

For example, participants in clinical trials have continued to use the product for a very long time, even now. Since this disease is progressive, it is generally expected that the medication will be prescribed for an extended period with the goal of slowing its progression as much as possible.

Innami [M]: Understood. Thank you. That is all from me.

Moderator [M]: Thank you very much.

Next, Ms. Hayase, please ask your questions.

Hayase [Q]: My name is Hayase from Mix. Thank you. I'd like to ask President Sonoda a question.

I understand you've set a goal of JPY100 billion in sales for the 2030s. To what extent do you expect givinostat to contribute to this goal, and what potential do you see for it? I would also appreciate it if you could share with me again your expectations regarding matters other than sales.

Sonoda [A]: Thank you for your question.

On the slides presented today, I showed that the figure was JPY35 billion. If we reach this point, and the target patient population trends higher, meaning figures beyond this may be expected, I believe that comparing this figure with the JPY100 billion target will make it clear that givinostat will contribute significantly to the JPY100 billion target.

We had already stated that we would aim for JPY100 billion even without givinostat. We had believed that we could achieve our goals sufficiently through the assets we currently possess, our globalization and technology licensing, but with the addition of givinostat, I believe our chances of success have increased, and we may be able to achieve our goals sooner.

Next, regarding the second question, I understood it to be asking whether there are any other benefits, or rather, whether there are any other positive aspects, resulting from the partnership with Italfarmaco. In that sense, as part of the givinostat agreement, both companies have agreed to conduct a comprehensive range of studies in the field of rare diseases.

Therefore, while it states here that they are working in the field of rare diseases, such as DMD and ALS, as listed here, we focus primarily on rare diseases as well.

We are, if anything, a research-oriented company that excels at research, while they are, if anything, a company that excels at sales. So, I'm currently thinking that we might be able to form a collaboration where our shortcomings and their shortcomings each other well, and I hope to pursue a collaboration going forward that allows both parties to maximize their strengths.

That is all.

Hayase [Q]: Thank you very much. Sorry, one more question.

I understand that clinical studies involving Japanese participants are also being planned. Could you please tell me what you can about the schedule?

Tanizawa [A]: Thank you for your question. This is Tanizawa.

As mentioned on the slides, since we have already submitted the initial Clinical Trial Notification, once it is accepted, we will be able to proceed with the contract. I'm thinking we might be able to post that information on the public disclosure website as well.

Hayase [M]: Thank you.

Moderator [M]: Thank you very much. Does anyone have other questions?

If there are no further questions, I'll go ahead and wrap up the question-and-answer session.

With that, we will now conclude the Givinostat Update of JCR Pharmaceuticals. Thank you very much for joining us today.

[END]

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