JCR Pharmaceuticals Co., Ltd.

Briefing Session on the Agreement with Italfarmaco Announced on December 24, 2025

January 23, 2026

Event Summary

[Company Name]	JCR Pharmaceuticals Co., Ltd.
[Company ID]	4552-QCODE
[Event Language]	JPN
[Event Type]	Special Announcement
[Event Name]	Briefing Session on the Agreement with Italfarmaco Announced on December 24, 2025
[Fiscal Period]	
[Date]	January 23, 2026
[Number of Pages]	26
[Time]	15:00 – 15:57 (Total: 57 minutes, Presentation: 15 minutes, Q&A: 42 minutes)
[Venue]	Webcast
[Venue Size]	
[Participants]	
[Number of Speakers]	5

Shin Ashida	Representative Director, Chairman, President, and CEO
Hiroyuki Sonoda Ph.D	Director, Senior Managing Executive Officer, Research, Executive Director, Research Division
Yoh Ito	Senior Executive Officer, Corporate Strategy, Executive Director, Corporate Strategy Division
Mathias Schmidt PD, Ph.D	Executive Fellow
Kazunori Tanizawa	Expert Fellow

[Analyst Names]*	Hidemaru Yamaguchi	Citigroup Global Markets
	Kazuaki Hashiguchi	Daiwa Securities
	Kota Maeda	Nomura Securities

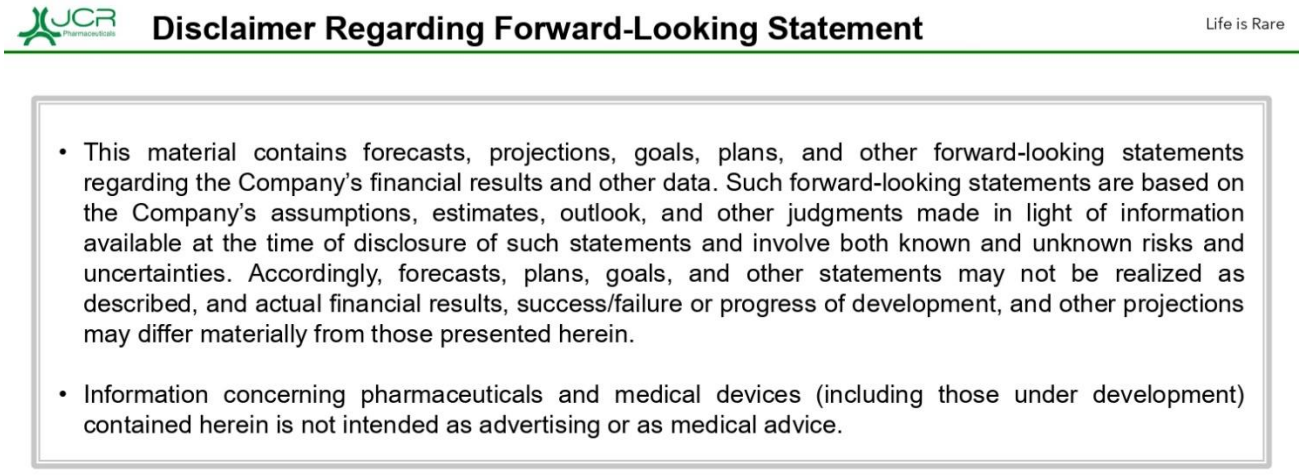
Miyabi Yamakita	Jefferies
Yuriko Ishida	Mizuho Securities

*Analysts that SCRIPTS Asia was able to identify from the audio who spoke during Q&A or whose questions were read by moderator/company representatives.

Presentation

Moderator: We would like to start the briefing on the agreement with Italfarmaco announced on December 24, 2025.

Scripts and videos of today's presentation and Q&A session will be available on our official website at a later date.



We would like to mention some points before the start of the briefing. In this presentation, we may make forward-looking statements based on our current expectations, all of which are subject to risks and uncertainties. In addition, today's presentation and the materials used are intended to provide information about our business to shareholders, investors, and the press. Information on developments and pharmaceutical products is not intended for the purpose of advertising or medical advice.

Here are today's speakers. Shin Ashida, Representative Director, Chairman, President, and CEO. Hiroyuki Sonoda, Director, Senior Managing Executive Officer, Executive Director, Research Division. Yoh Ito, Senior Executive Officer, Executive Director, Corporate Strategy Division. Mathias Schmidt, Executive Fellow. Kazunori Tanizawa, Expert Fellow. These five members.

The materials to be used today were posted on our website at 2:30p.m.(JST) today. We apologize for the inconvenience, but if you need the documents at hand, please refer to them.

Today's presentation will last approximately one hour, including Q&A. Questions will be collectively answered after the presentation. The Q&A session will last approximately 40 minutes.

Chairman Ashida, please go ahead.

- **Development and Commercialization of Givinostat for DMD**
  - Exclusive licensing agreement (Japan)
- **Strategic collaboration for the treatment of rare diseases**
  - Enhancing both companies' portfolios
  - Exploring joint opportunities across JCR's R&D pipeline and platform technologies

2

Ashida: I'm Ashida. Thank you very much for joining us today.

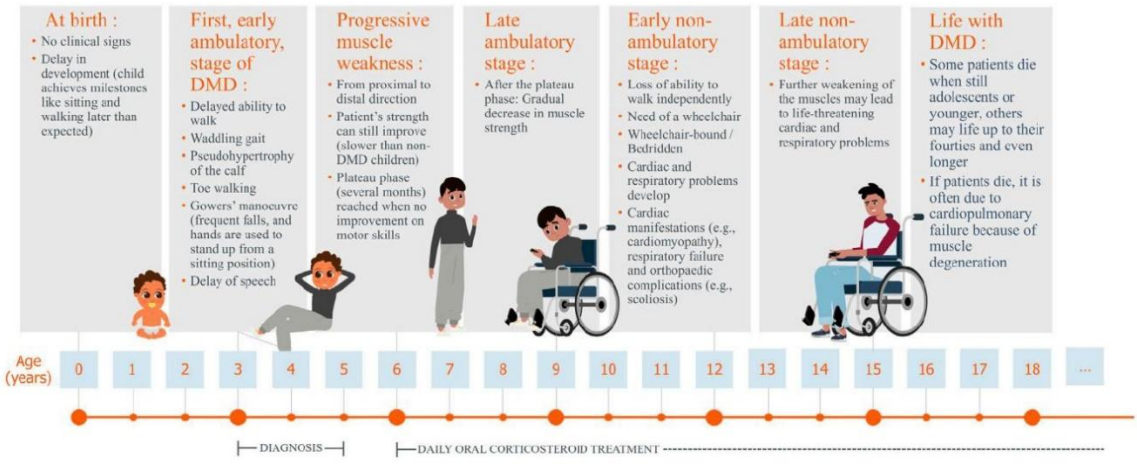
Today, we would like to talk about our acquisition of the rights to market Duchenne muscular dystrophy (DMD) drug Duvyzat of Italfarmaco in Japan. For this Duvyzat, Italfarmaco has already taken the manufacturing and marketing rights in the US and Europe, and has been marketing the product in the US for a year and a half, acquiring 20% of the Duchenne muscular dystrophy market.

Considering the growth hormone and other products that we are currently selling, we believe that this formulation will bring in significant sales and profits for JCR in the future.

Today, I would like to briefly explain the background of Duvyzat, its position in our product lines, and our future approach to Duvyzat. Thank you very much.

Overview: Duchenne Muscular Dystrophy (DMD)

Life is Rare



DMD, duchenne muscular dystrophy
Desmet T, et al., *Front Pharmacol.* 2025; 16: 1662586.

4

Tanizawa：From here, Tanizawa will give an overview of the newly introduced Givinostat. Thank you very much.

DMD is a progressive, severe, and rare disease of childhood onset, and is usually diagnosed between the ages of 3 and 5. It is a serious disease that progresses to the point where the child cannot walk by the age of about 10.

Overview: Duchenne Muscular Dystrophy (DMD)

Life is Rare

1 Etiology[1]

- Caused by mutations in the dystrophin gene, leading to the absence of dystrophin protein beneath the muscle fiber membrane

2 Patient population[2]

- approx. 3,500 (Japan)

3 Clinical course[1]

- Onset at 3–5 years of age
- Progressive motor decline; loss of ambulation around 10 years
- Respiratory and cardiac complications thereafter
- Inter-individual variability in progression

4 Treatment landscape

- Only two therapies approved in Japan (excluding steroid therapy)
- Drug lag and drug loss remain key challenges for treatments approved by the FDA and EMA

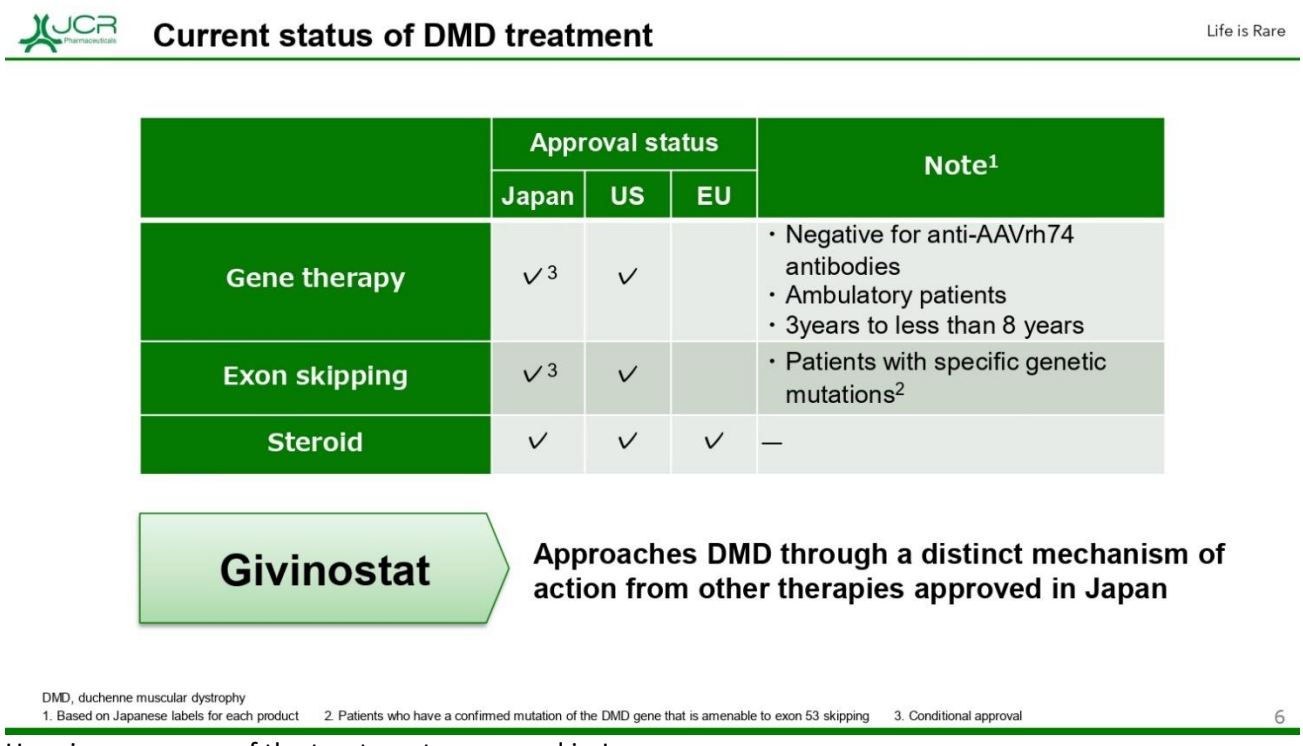
DMD, duchenne muscular dystrophy
1. Clinical practice guidelines for Duchenne Muscular Dystrophy 2014 (Japanese) 2. Kawai M. *No To Hattatsu.* (Japanese) 2013;45(Supple.):S324

5

The etiology is a mutation of the dystrophin gene and a deficiency of dystrophin protein, and there are approximately 3,500 patients in Japan. Symptoms, as I mentioned earlier, include the inability to walk at

around 10 years of age, and after that, respiratory failure and cardiomyopathy are very important factors in the prognosis.

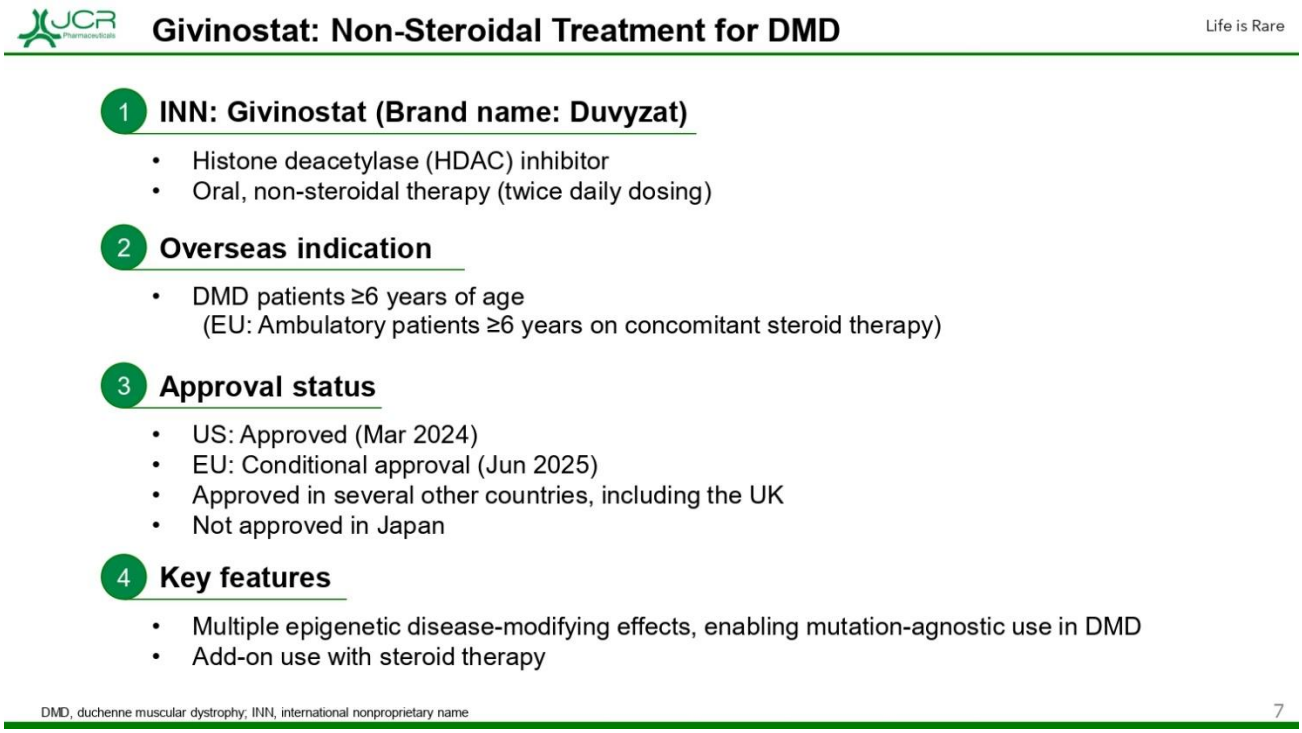
In Japan, two formulations have been approved for the dystrophin gene approach. This excludes steroid preparations. According to what I have heard from doctors and patient groups, the drug-lag loss, which is caused by the fact that the drug has already been approved by the FDA and EMA but not approved in Japan, is still a very big issue in this area. We would like to make a contribution in this regard.



Here is a summary of the treatments approved in Japan.

There are gene therapy and exon skipping therapy: as you can see on the right side of this page, gene therapy is limited to patients between 3 and 8 years of age. Exon skipping is also limited to patients with specific genetic mutations, so I would say that about 80% of DMD patients in Japan have no treatment at all at this point. We believe this is a very important point.

Givinostat has a different mechanism of action than other drugs approved in Japan, and is expected to be widely applicable to patients with DMD.

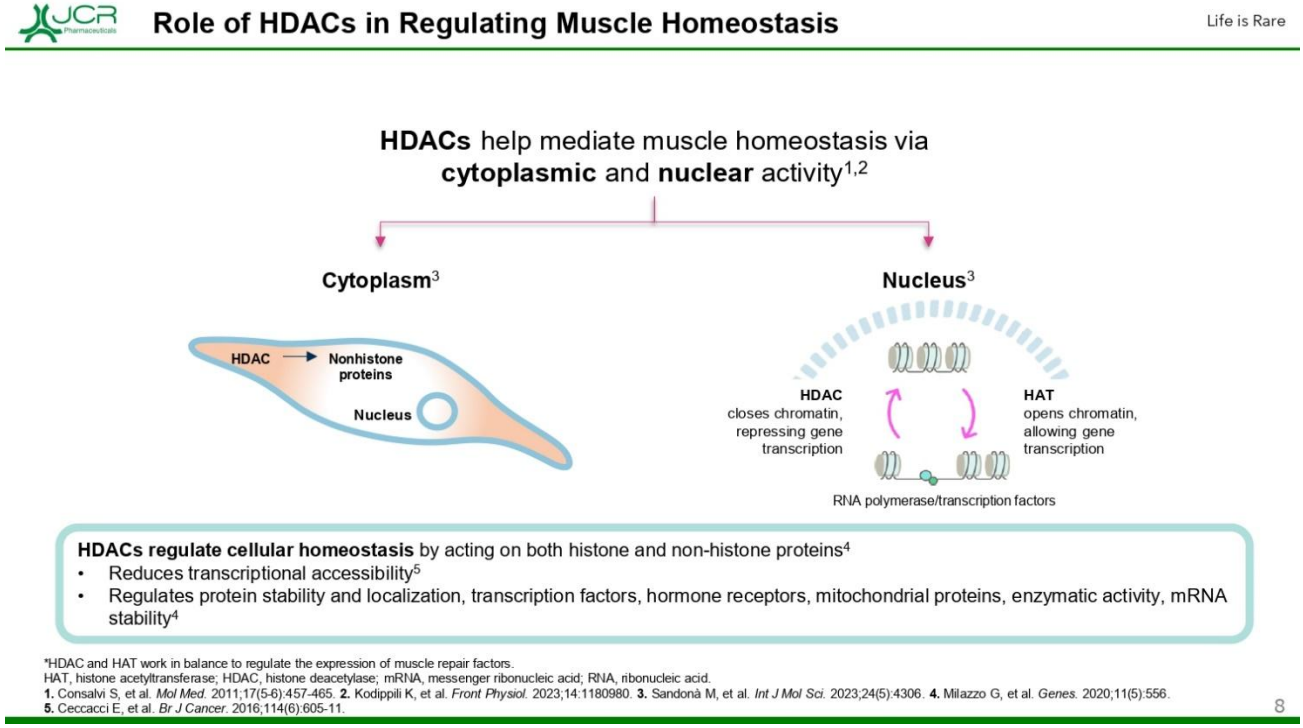


Here is a brief overview of Givinostat.

The mechanism of action is described as an inhibitor of histone deacetylase, or HDAC inhibitor. It is administered orally and is designed to be taken twice daily.

The overseas indication is for patients with Duchenne muscular dystrophy aged 6 years and older in the US no genetic mutation is specified. In the US, approval was granted in March 2024, and in the EU, conditional approval was granted in June 2025.

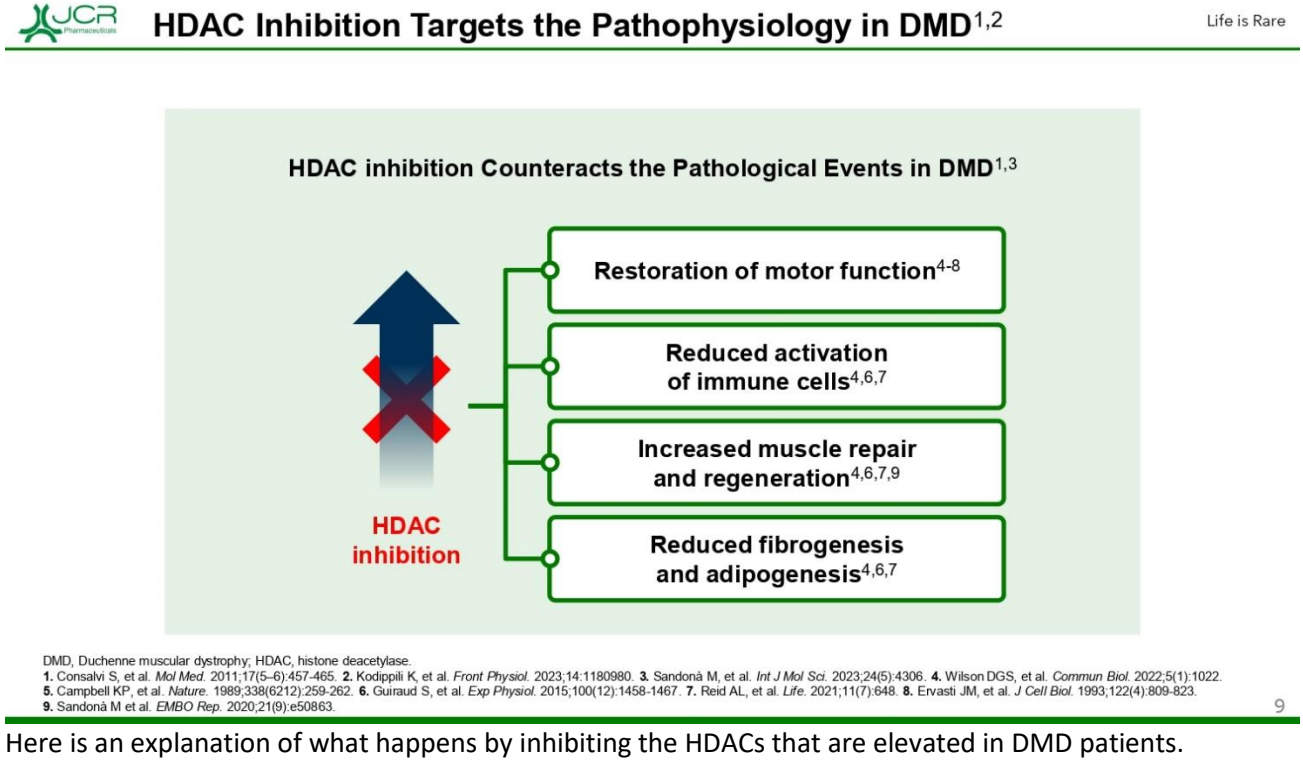
Finally, it is characterized by its multiple epigenetic disease-modifying actions, which make it possible to use it independently of the genetic mutations of DMD patients. We assume that it will be used in combination with steroids.



As mentioned earlier, HDAC is a histone deacetylase, which itself acts on histones in the nucleus to regulate gene expression.

On the other hand, non-histone proteins, which are located in the cytoplasm on the left side, also have many effects, and by approaching these proteins, muscle homeostasis is regulated.

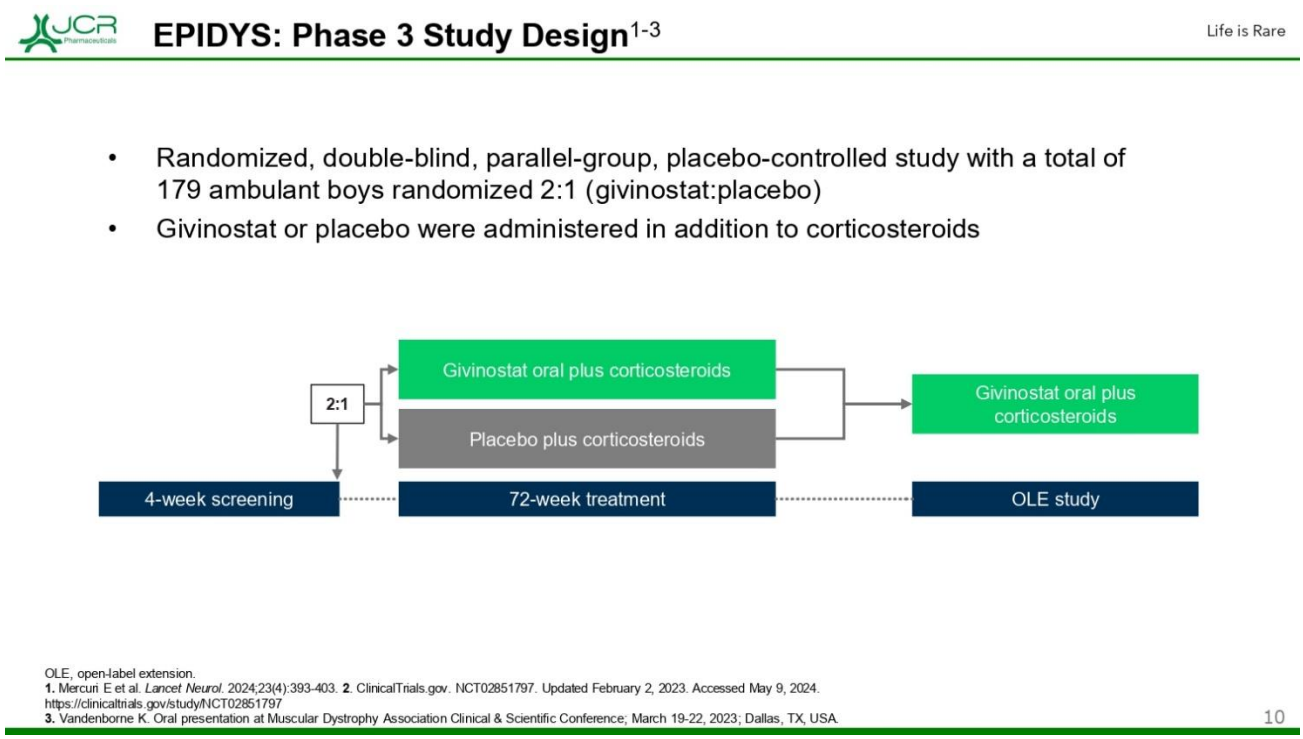
However, it has been reported that the activity of HDACs is greatly increased in DMD patients, resulting in inhibition of muscle regeneration and increased inflammation.



Here is an explanation of what happens by inhibiting the HDACs that are elevated in DMD patients.

As I explain from the bottom right, first of all, suppression of fibrogenesis and adipogenesis is expected. In addition, the promotion of muscle repair/regeneration, the suppression of inflammation, and the suppression of activation of immune cells are expected to occur as epigenetic disease modifications, resulting in the eventual recovery of motor function in muscular dystrophies.

This content has been examined in a clinical trial, and I would like to explain the results of the clinical trial next.

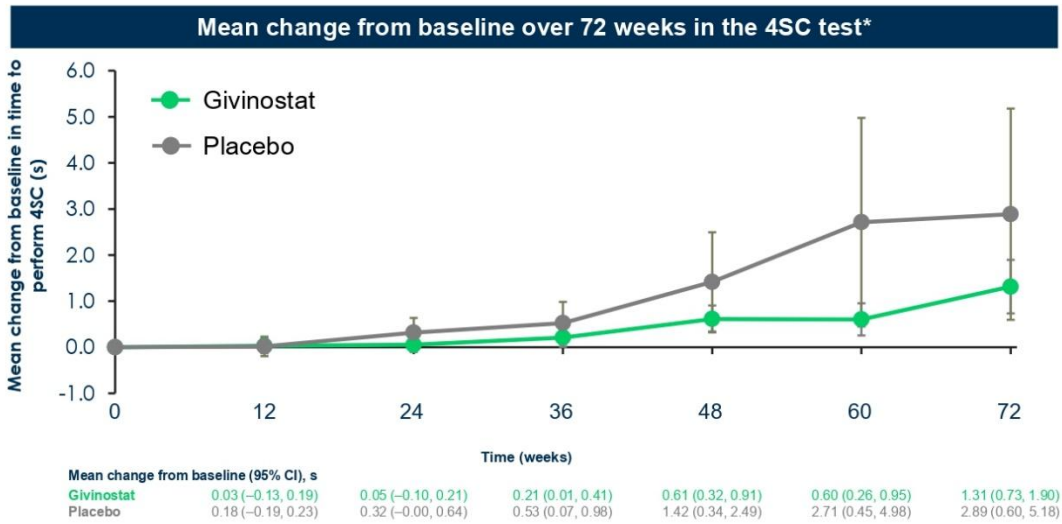


There are many clinical trials being conducted, but this is a global Phase III clinical trial, a validation study design.

179 ambulatory patients were recruited, randomized 2:1 to Givinostat or placebo, and the trial duration was 72 weeks, followed by extension study.

EPIDYS Study: 4SC From Baseline to Week 72[1]

Life is Rare



*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; s, seconds.
1. Mercuri E et al. *Lancet Neurol*. 2024;23(4):393-403.

11

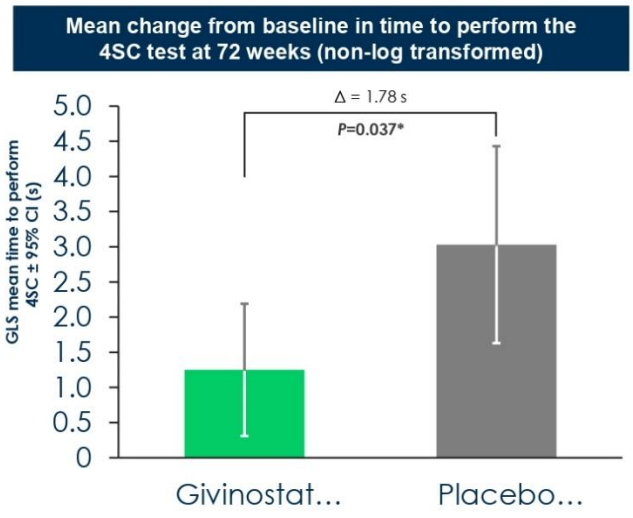
Here are the main results. It says 4SC, which measures the time to climb a 4-step staircase.

Compared to the placebo group, you can see that the disease progression was suppressed at all-time points in the Givinostat administration group.

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.

12

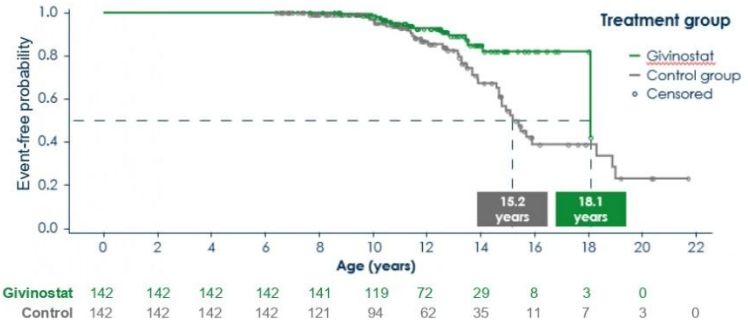
In the primary endpoint, placebo and Givinostat at 72 weeks were compared.

The results were also very clear, and the difference was statistically significant. As for time, the improvement was 1.78 seconds.

Givinostat and Permanent Loss of Ambulation[1,2]

Life is Rare

- Patients receiving givinostat plus corticosteroids (SoC) preserved their ability to walk for an additional 2.9 years (HR, 0.42; 95% CI, 0.23-0.76; *P*=0.004) compared with patients receiving SoC alone



Parameter	Givinostat (n=142)	Control (n=142)
Patients, n (%)		
Assessed	142 (100)	142 (100)
Who lost ambulation	14 (9.9)	39 (27.5)
Censored	128 (90.1)	103 (72.5)
Age at loss of ambulation, years		
Median (95% CI)	18.1 (18.09, NE)	15.2 (14.70, 18.31)
P-value	0.004	
HR (95% CI)*	0.42 (0.23, 0.76)	

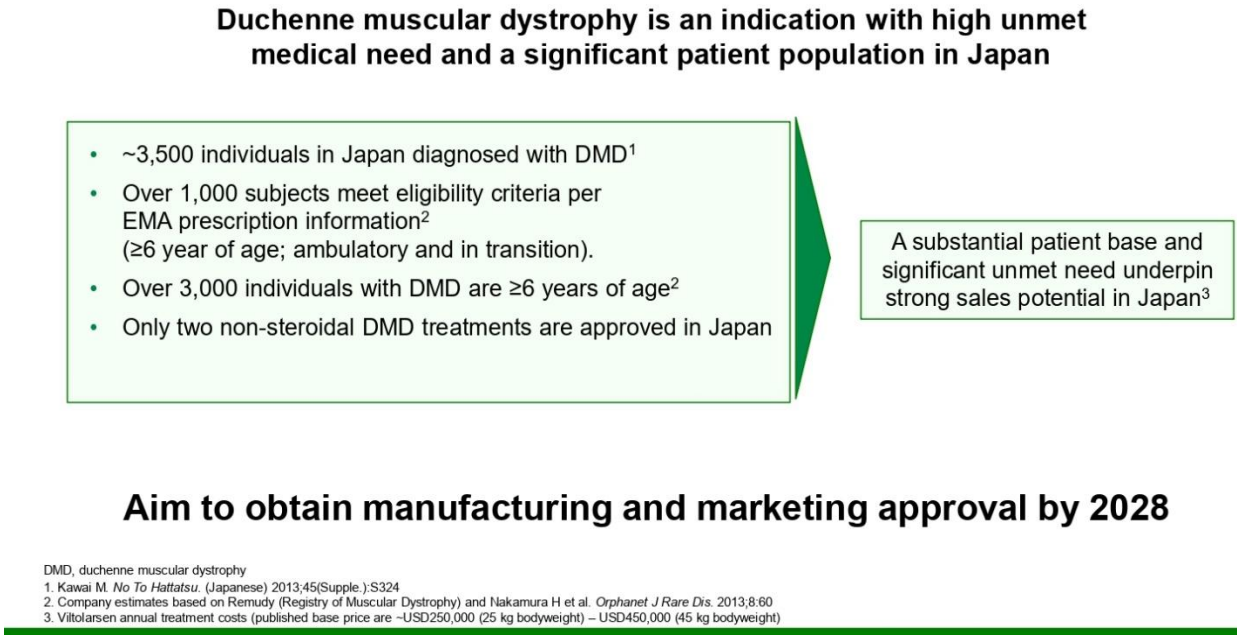
*HR and associated 95% CI and *P* value are obtained from a Cox proportional hazards model, including the treatment group as an independent classification factor.
HR, hazard ratio; NE, not estimable; SoC, standard of care.
1. McDonald CM, et al. *Ann Clin Transl Neurol.* Published online August 19, 2025. 2. Post hoc analysis comparing with natural history disease studies, using data that including the EPIDYS study

13

This is another important piece of supportive evidence, examining the effect of Givinostat on the maintenance of ambulation ability.

The green line is the actual drug, Givinostat group, and the gray line is the natural history, patients with matched backgrounds.

As a result, the Givinostat group maintained their ambulation ability for 2.9 years longer than the other groups, so it is expected that early treatment with this drug will be effective in maintaining ambulation function as long as possible.



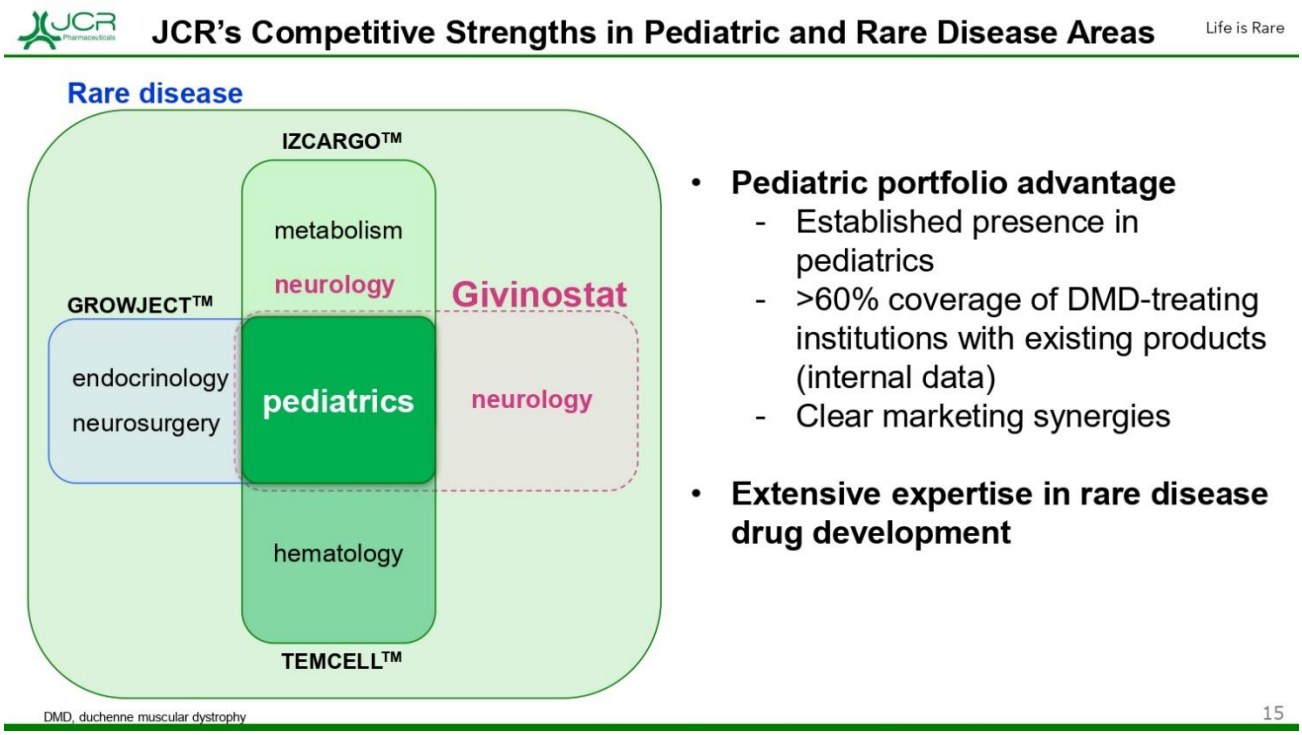
From here, I would like to explain the potential of Givinostat in Japan.

As I mentioned earlier, we have been receiving requests from doctors and patients for a solution to this drug-lag loss, and since about 80% of DMD patients have no treatment at all, I believe there is great potential in this area.

Specifically, there are 3,500 DMD patients in Japan, and more than 3,000 are over the age of 6. Even if we break it down into patients who are at least 6 years old and ambulatory, there are more than 1,000 patients, so you can imagine how much patients are counting on this drug.

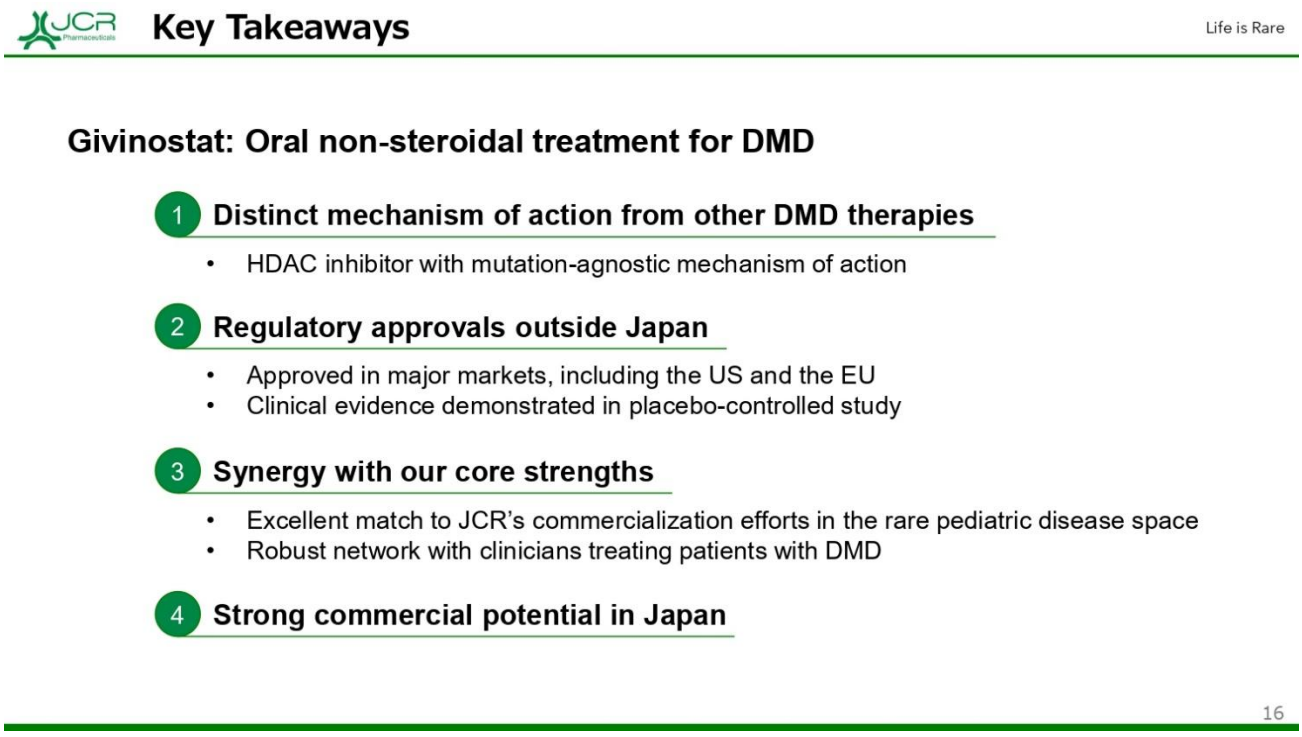
One more thing, the drug-lag loss is in progress, and we have already received approval from the FDA in 2024 and from the EMA in 2025. I believe that our biggest contribution should be how quickly we can obtain approval in Japan.

Since this is a rare disease in children, we would like to aim for manufacturing and marketing approval by 2028 with our experience in this area.



JCR Pharma has been working on its own products in the field of pediatric and rare diseases. As a result, we have strong partnerships with doctors and hospitals in the field of pediatrics. This is directly linked to the cooperation with doctors and hospitals that follow DMD, and I believe that more than 60% of facilities are covered at this point.

The other, as I mentioned earlier, is our experience in development in the area of rare diseases to prevent drug-lag loss. We would like to take advantage of it as well, while aiming for approval as soon as possible.



This is the last slide.

Givinostat is an oral, non-steroidal treatment that is independent of genetic mutation. This is the point of today's presentation.

Overseas, it is approved in the US, Europe, and other major countries, and the evidence is strongly shown in placebo-controlled clinical trials.

I think we have our strengths, compatibility with the pediatric and rare disease areas, and a strong network of DMD physicians.

As I mentioned at the end of this presentation, we recognize that this is a drug with great potential and that it is highly anticipated.

That is all from me.

Question & Answer

Moderator [M]: We will now move to the question-and-answer session.

We will take questions first from analysts and then from the media.

Mr. Yamaguchi, please start with your question.

Yamaguchi [Q]: I'm Yamaguchi from Citi.

The first question I would like to ask is regarding the contract, although it is not in the release. There seems to be little disclosure on how much you pay and what the milestones are, or whether there are royalties in the future, but is it possible to provide the information necessary to make some kind of earnings forecast?

Ito [A]: My name is Ito.

We would appreciate your understanding that we are unable to disclose the details of this contract or the amount of the contract due to an agreement with the other party.

However, I am sure that you can also estimate how much sales are likely to be based on what I have explained this time, and I would appreciate your cooperation in this regard.

Yamaguchi [Q]: This seems to be an unlisted company, do you know how much sales in the US are now?

Ito [A]: Again, Italfarmaco is a private company, so we are not allowed to disclose the amounts, etc. We apologize for the inconvenience.

Yamaguchi [Q]: Do you know the drug prices in the US? There is a lot of information out there, though I think with muscular dystrophy, it is often in the tens of millions of yen.

Ito [A]: I think that is correct in your assumption.

Yamaguchi [Q]: Second, regarding the development schedule, I think there have been a few cases recently where development in Japan is kept to a minimum using drag-lag, etc., and those developed overseas are put in quickly, in some cases without testing. How long will the clinical trials in Japan be, and I am sure you are still in the process of consulting, but your company's assumption is that the product will be approved in 2028, which means that you will have to apply for approval in 2027 to make it in time, so it will be next year. What do you think about it?

Tanizawa [A]: I'm Tanizawa.

As you mentioned, there are various guidelines for pediatric and rare diseases that are highly needed in Japan, and we would like to plan clinical trials while referring to such guidelines.

As you mentioned a little, we would like to discuss with the authorities in the future how to minimize or implement what is necessary.

Yamaguchi [Q]: Taking those into consideration as well, you mean approval by 2028, right?

Tanizawa [A]: Yes. That is our goal.

Moderator [M]: Mr. Hashiguchi, please.

Hashiguchi [Q]: I am Hashiguchi from Daiwa Securities.

The first is what efficacy can be expected from this mechanism? What are your thoughts on this, other than what is now being shown in clinical trials?

I would also like to know what kind of clinical research is being conducted by Italfarmaco and other research institutions, and what kind of data we can expect to see in the future.

If HDAC hyperactivity varies from region to region within skeletal muscle. Also, I would like to know how HDACs are enhanced in DMD patients in the myocardium and what effect you think this HDAC inhibitor can have on the deterioration of cardiac function.

Tanizawa [A]: I'm Tanizawa.

The mechanism of action is very distinctive. It is said that in terms of muscles, it has influence on HDAC class 1 and 2. Specifically, the HDAC inhibitor is said to act with respect to about 1, 2, 3, 4, 6, and 8.

Since the genes regulated by each of them are different, I cannot give you a complete answer for each of them at this time, but in that sense, I believe that they work on a wide range of muscle regeneration.

Cardiac function is also very important, and naturally, this is a key point in muscular dystrophy, but I am not aware of any clinical trials currently underway that are evaluating myocardium.

Another question is what clinical trials Italfarmaco is conducting now and what data will be available later.

This has also been the subject of several clinical trials for DMD. At large, we are now conducting placebo-controlled clinical trials in non-ambulatory patients. The test itself is expected to be completed around 2027, so I think we can expect additional submissions of this data in Japan.

The other is that the drug is currently available for patients 6 years of age and older, but we are also conducting a parallel study for patients younger than 6 years of age, targeting younger patients. This will take a little longer, but we are in the process of acquiring data for 2029.

Hashiguchi [Q]: Just to confirm, you said that the effect of improving cardiac function or preventing worsening of cardiac function has not been evaluated in clinical trials up to now?

Tanizawa [A]: Yes. It needs to be confirmed, but it has not been evaluated with respect to what we are looking at in the main part of the current project.

The reason for this is that we are conducting clinical trials only with subjects who are able to ambulate. In other words, this will inevitably lead to younger patients. Since the evaluation of cardiac function inevitably comes after the subsequent assessment, I think there is a situation where it may be difficult to evaluate the effectiveness.

Hashiguchi [Q]: Secondly, I would like to know what the two lines in the fourth paragraph of the press release are trying to say.

Could you please comment a little more on what kind of developments you expect in the future in terms of establishing a broad strategic alliance between the two companies to explore joint opportunities in the treatment of rare diseases?

Sonoda [A]: Sonoda would like to answer.

Regarding this, as Tanizawa just mentioned, this Givinostat, of course, we are working together now, but also in other areas.

As you all know, JCR is very strong in biologics and mainly develops drugs for rare diseases. Italfarmaco's strength is in a slightly different field than ours, namely, in non-biologics, such as Givinostat, which is a small-molecule compound. That is their strength.

So, I am thinking that each of us can do various things jointly in a reciprocal and complementary manner. That is why we have written this statement, to say that together we will find new things again and explore opportunities for joint research.

Hashiguchi [Q]: It is more like looking for something you can do together in the area of drug discovery research, and the development and sales of what your company is currently looking for partners for at the late stage is not a high priority at present. Is my understanding correct?

Sonoda [A]: Well, both of them. We are not putting a break there, or making any difference, but are in the process of exploring various possibilities together.

Moderator [M]: Mathias, regarding this question, do you have any other additional comments from your side?

Schmidt [M]*: No thank you at this stage, no further comment from my side.

Moderator [M]: Mr. Maeda, please go ahead.

Maeda [Q]: I am Maeda from Nomura Securities.

I had an understanding that you would take advantage of your company's sales base to expand to some extent into this DMD area. By expanding into the area of DMD this time, it seemed to me that you are creating a situation that will make it easier for your company to take on more and more challenges in DMD in the area of gene therapy in the future. I would appreciate it if you could comment on whether that interpretation is, in fact, correct.

Sonoda [A]: Sonoda will answer the question.

As you mentioned, we are developing a new AAV vector called JUST-AAV, which has improved delivery to the CNS and muscle tissues, so it is conceivable that muscle diseases, including DMD, could be targeted for gene therapy.

But on the other hand, regarding the introduction of Givinostat this time and whether it is directly linked to this, it is absolutely not the case that we did this because of that. As I mentioned earlier, we are now working on something that has the potential to first fill this drug-lag loss in Japan.

We are considering the possibility of having what you have just mentioned as an extension of this, but it is also very much related to our strategy, of which indications and clinical PoCs we will use for JUST-AAV, so I don't think that we should start with DMD genes just because we have introduced this.

Moderator [M]: Mr. Yamaguchi, please go ahead.

Yamaguchi [Q]: Two additional points, briefly, please.

A drug-lag issue was mentioned. I'm sure there will be many more of these in the future, but as long as they are rare and in the pediatric setting, do you think that you will continue to take them in the future, regardless of modality or disease? Or is it more like this one was taken because it seemed to work, in many ways, rather well?

Ashida [A]: Ashida will answer.

We don't mean we are taking out those with drug-lag issues, but we would like to do what we can in the field of rare diseases, if there is any.

Yamaguchi [Q]: That is the case, right? So it just happened to be a drug-lag drug.

Ashida [A]: That's right.

Yamaguchi [Q]: Secondly, this is a little more detailed, but this is sold in bottles, and the amount used varies, but would it be correct to think of this as using one bottle a month?

Tanizawa [A]: I would like to confirm that. We do this by taking the required amount in a syringe and taking it twice a day. That depends on the weight as well.

Moderator [M]: Mr. Yamakita, please go ahead.

Yamakita [Q]: My name is Yamakita from Jefferies.

I see that you have stated that you have covered more than 60% of the follow-up facilities for DMD patients in the sales of your existing products. Will you be investing in sales to get the remaining 40%, and this as well? This is because I think your company also has new technology for myotropic technology, I think. I wonder if there is an incentive to expand for muscular dystrophy specialists in those areas as well. The question is, will you make a solid investment, or in short, will you spend the cost?

Ito [A]: Ito will answer the question.

As Chairman Ashida explained at the beginning of this presentation, we expect Givinostat to have a very large commercial potential. From this standpoint, we will invest in sales and marketing, including human resources, in order to maximize the spread of this drug.

Yamakita [Q]: I would like to ask the second question.

I think it was around June of last year that Mr. Sonoda mentioned that he had started visiting the partner directly for meetings regarding out-licensing projects. Regarding this in-licensing, how would you describe your company's R&D members, the members of BD, and their involvement in the project? What is your comment?

Sonoda [A]: Sonoda will answer the question.

We do not have any special rules regarding which department must do which in the in-licensing, out-licensing, and technology introduction areas.

But on the other hand, there are various talks at events like BIO US, BIO EU, and other such events where general biotech companies are present. At that time, of course, there is talk of in-licensing, out-licensing, and various other discussions that take place there. We are picking up a variety of information from these sources, including the members present at the conference and myself, and we are responding on a case-by-case basis to those that we think can be in-licensing or put-licensing from such information.

Moderator [M]: Mr. Hashiguchi, please go ahead.

Hashiguchi [Q]: I'm Hashiguchi from Daiwa Securities.

I believe Italfarmaco is conducting a Phase III study of this drug for polycythemia vera. What are your thoughts at this time on the possibility of developing this indication in Japan as well? If you are interested, what are your plans now as to when you will launch it?

Tanizawa [A]: I'm Tanizawa.

As for the HDAC inhibitor Givinostat, clinical trials have been conducted for many indications, as you mentioned. The first indication approved was the DMD, Duchenne type. Another thing I would like to mention is that the clinical trials have been conducted for the Becker type, and the same mechanism of action is expected.

As for your question about polycythemia vera, clinical trials have been conducted for a long time, and I think it may be necessary to discuss various issues once we have obtained approval, but at this stage, the agreement includes the development of DMD in Japan, so we will consider that in the future when the time is right.

Hashiguchi [Q]: So, contracts are currently limited to DMD?

Tanizawa [A]: Yes. That is the way for the in-licensing contract.

Hashiguchi [Q]: I think that in the past there have been clinical trials for various indications, but many of them have not been active, but are polycythemia vera and Becker's type active in one way or another? The Becker type seems to take quite a bit of time, though.

Tanizawa [A]: I would say that the DMD was the first one that finally got approval, but as you said, with the understanding that various things are being considered, including applications. We do not have accurate information on this issue at this time.

Moderator [M]: Ms. Ishida, please go ahead.

Ishida [Q]: I am Ishida from Mizuho Securities.

I wonder if clinical trials will be added because of the DMD case this time, but how should we think about the cost of R&D expenses?

Tanizawa [M]: Do you mean the size of the cost, or which side will share the cost?

Ishida [Q]: As well as which side will share the cost, I would like to get some sense of how much it is likely to add to the R&D costs, or some sense of what the R&D costs will look like.

Tanizawa [A]: This is really another story that will be told in the future, and I think the biggest point will be feasibility. Since DMD is still a rare disease in Japan, it is not feasible to conduct a 179-patient trial in Japan as indicated earlier, nor is it possible to conduct a placebo-controlled trial.

Looking at the number of patients in other clinical trials, I am not sure if there are that many patients, say 100 patients. I cannot give you a specific number of patients, but I assume that the cost of the clinical trial will not be that large.

Moderator [M]: I would like to include the media in the questions.

Ms. Narita, please go ahead.

Narita [Q]: I am Narita from the Nikkan Yakugyo.

I would like to make one quick point, but you mentioned earlier that you were aiming for approval by 2028. Is it correct that the timing of the application has not yet been decided?

Tanizawa [A]: We are setting a goal right now, which is by 2028. Of course, this disease is a rare disease, so we are considering submitting an application with a timeline that also assumes that it is an orphan drug. However, we have not yet finalized when the application will be filed.

Moderator [M]: Ms. Tsubokura, please go ahead.

Tsubokura [Q]: This is Tsubokura from The Chemical Daily.

What I would like to ask you is that you are going to consult with the authorities in Japan. Do you start the consultation under the same conditions as the United States, which has a large patient population?

Tanizawa [A]: Tanizawa will answer the question. I think that understanding is correct.

Tsubokura [Q]: I would also like to ask how much market share you are aiming to achieve for the treatment of DMD in Japan. I think the number of patients will vary depending on what kind of indications the drug is close to, but please let me know if there is something you are aiming for in terms of percentages.

Tanizawa [A]: We don't have any target values in the share with the percentage at the moment, but as I explained earlier, we believe that the patients for whom this formulation is indicated are very wide. For example, even if we limit the number of patients to those who are at least 6 years old and ambulatory, there are more than 1,000 patients, and we would like to explain the evidence so that as many of these patients as possible can use the drug.

If the drug could be used regardless of walkability, for example, I think our goal would be changed.

Tsubokura [Q]: One more thing, at the beginning you mentioned that the drug has about 20% share of DMD market in the US, and as for the reason for this figure. It seems a bit small, but could you tell us what kind of competitive environment you think led to this kind of figure?

Tanizawa [A]: The approval in the US was in March 2024. The situation is now less than two years. The fact is that one in five patients uses the drug, and we recognize that the drug has been penetrating into the market quite quickly. We are also aware that this may not be the peak.

Moderator [M]: Mr. Hashimoto, please go ahead.

Hashimoto [Q]: I'm Hashimoto from Nikkei BP.

There was a question earlier about the part of the press release where it says strategic alliances in the field of rare diseases. In your earlier explanation, I think you said that you are going to explore various possibilities. I am sure that you are considering such a partnership not only with this company, but with various other companies as well, but I would like to know the purpose of writing this, or perhaps you are saying that this company in particular has synergies with your company, or that it is attractive to you, and that you will form such a strategic alliance with this company in particular. If there is, I would like to know about it.

Sonoda [A]: Sonoda would like to answer.

This time, I think there are various diseases and target tissues among the rare diseases. We also had a question earlier about having directional vectors in the muscles.

We have been developing technologies that can be delivered to the CNS and muscles, for example, but we need to work with companies that are familiar with the diseases that affect the CNS and muscles, and have the knowledge of how our technology can be used for those diseases, we believe that working together with companies that are familiar with the disease and have the knowledge and know-how of how our technology can be used in the disease will lead to faster development and better products.

We may also be able to combine with other modalities that we do not have, for example, in the future. Of course, Italfarmaco is not the only company that has it, but Italfarmaco has it. Since we have reached this kind of partnership, I think we can expand from there and do a variety of things.

Hashimoto [Q]: So, is it my understanding that you think there is a possibility to utilize vectors that target muscle, such as the J-Brain Cargo mentioned earlier, in the diseases targeted by Italfarmaco?

Sonoda [A]: Yes. In part, I believe there is a good chance of that.

Moderator [M]: Ms. Hayase, please go ahead.

Hayase [Q]: I'm Hayase. My question may overlap with others a bit.

As a research and development-oriented company, we understand that your company's strength lies in in-house drug discovery. I would like to ask again how the in-licensing of Givinostat fits into your company's R&D-based business model and overall drug discovery strategy.

Ito [A]: Ito would like to answer the question.

I am sure you can understand that our current profit/loss situation is not that stable.

This means that the gross profit from domestic product sales cannot easily cover all of the R&D and SG&A expenses. So what kind of structure is it? It's a structure where profits are generated by including lump-sum payments from contracts or milestones arising from existing joint research projects, among other such sources. Contract income is not always stable, since there is a partner to deal with.

We have been thinking about various ways to change this unstable structure to a stable one as soon as possible, and to bring it to a state where all such expenses can be covered by the gross profit from the sales of products that can be reliably expected, and change can be made as quickly as possible.

In this context, we were able to introduce a product with this very promising commercial potential. By doing so, we would like to increase sales and profits over the next few years, and invest the money we make in research and development, the current J-Brain Cargo and JUST-AAV, as well as the next generation of products, and to invest money to bring them out for the future. As part of this process, we decided on the in-licensing.

Therefore, we want to increase sales with this product, to keep that kind of style, which is solely R&D oriented. That is the way we think.

Moderator [M]: Mr. Okada, please go ahead.

Okada [Q]: My name is Okada from Yakuji Nippo.

Just to be sure, have you decided to conduct clinical trials? Do you start by considering whether or not to implement a study? To begin with, the 179-patients clinical trial, are there no Japanese in here? Can you please tell us about these points?

Tanizawa [A]: Tanizawa will answer the question.

Consultation with the authorities will be starting at full scale soon, and we will be considering various methods, as you have just mentioned.

Specifically, they include that if we will not conduct clinical trials, or we will conduct small-scale trials, or we will conduct what is necessary. These are the possible patterns.

As you mentioned about Japanese data in the global clinical trial, there are no Japanese participants, but we have six Asian participants, so I think such data, efficacy and safety data, and differences in PK, etc., will be very important data.

Okada [Q]: You explained at the beginning, but could you please explain again the differences and differentiation from the existing products approved in Japan?

Tanizawa [A]: Currently, there are two treatments approved in Japan other than steroids: gene therapy and exon skipping therapy.

A major point of differentiation, or perhaps a better way to put it, is the difference in indication. There is a provision that gene therapy is limited to patients between 3 years of age or more and less than 8 years of age. Exon skipping is defined as a therapy for patients with a specific genetic mutation.

On the other hand, in terms of the approval we are aiming for, the product can be used for patients aged 6 years and older, so I think this difference can be explained.

Mechanism of action in this gene therapy, exon skipping is an approach to the Dystrophin gene, and as for Givinostat, it is a disease modulator, modifier, or inhibitor of HDACs, and can be widely used.

Finally, on a factual basis, the fact that Givinostat has been approved in the US and Europe makes this drug different from other approved drugs. As you can see here, the situation is that for two other drugs, they are approved in the US, and as for the EU, Givinostat is approved.

I think the other part that could be explained is the results of clinical trials and such.

Okada [Q]: If it is over 6 years old, how old can the oldest one be?

Tanizawa [A]: The regulations only refer to the age of 6 and up, so there is no limitation on the oldest.

Okada [Q]: So one differentiator would be to be able to handle 9 or even early 10-year-olds, since they are likely to have difficulty walking at about 10 years of age?

Tanizawa [A]: Yes, you are right.

Moderator [M]: Okay, since Mr. Imazu has already asked a question via chat, I will read it for him. This is a question received from Mr. Imazu of Yomiuri Shimbun.

Imazu [Q]: The first question is that it affects histone modifications, what side effects have been reported from this? Is the frequency clear?

Tanizawa [A]: In this regard, there have been reports of thrombocytopenia and elevated triglycerides as side effects specific to HDAC inhibitors. These are already included in the US and European drug inserts as adverse events that should be watched with particular attention.

However, as for the risk of thrombocytopenia, it was considered that there is no increased bleeding tendency, for example, and the drug was approved based on its risk and benefit. I think that is the point we need to pay attention to in our clinical work.

Imazu [Q]: The second question. Am I correct in understanding that this is expected to work for Becker type as well as DMD?

The understanding of the premise is that existing therapeutics use an approach that compensates for frame shifts by exon skipping, and that is how nucleic acid drugs or gene therapies are approved. It is my understanding that this case is not a direct gene action, but rather an action on cytoplasmic proteins that reduces inflammation in the muscle and suppresses the progression of symptoms.

I would appreciate an answer along with the effect on the Becker type as well as DMD.

Sonoda [A]: Sonoda will answer the question.

As a general theory of the mechanism of action, I believe that it can be expected to be effective enough for the Becker type as well.

In response to what was said earlier, the cytoplasm, there is also that, but the biggest thing is that when this HDAC is hyper activated, it turns off the gene switch. The genes are packed so tightly that they cannot open slowly and express themselves as they normally should. In other words, the switch is forced to be off.

It is turned off, and many bad things happen, and it also acts on proteins in the cytoplasm, which are deacetylated just like histones. We can say that these two actions cause bad things.

But the action of unpacking genes and allowing them to express what they should be expressing is very significant. So, considering this mechanism of action, I would say that it can be expected to be effective enough for similar muscle diseases other than DMD, or as you asked, other diseases where fibrosis is a problem.

Moderator [M]: If there are no questions, we will now conclude the question and answer session. Mr. Mathias, is there anything to add?

Schmidt [A]*: Thank you. Two comments to two questions.

There was a question about the R&D costs. And what I would like to mention is the cost for the clinical trial. Will be borne by Italfarmaco up to an agreement cap.

The second question was about the cardiovascular efficacy of Givinostat.

While the efficacy on the heart itself was not independently measured, what I would like to say is that the four step climb test is something that is highly relevant for the patients and actually requires both, a functional heart and it requires a functional muscle.

And, just as a reminder, this endpoint was met with clinical statistical significance - the difference to the placebo-control.

The difference that you see is absolutely clinically relevant.

Thank you very much.

Moderator [M]: Since there seems no question anymore, we would like to end the question-and-answer session.

This will conclude the briefing on the contract between JCR Pharmaceuticals and Italfarmaco published on December 24, 2025. Thank you very much for your participation.

[END]

_____

Document Notes

1. *Portions of the document where the audio is unclear are marked with [inaudible].*
2. *Portions of the document where the audio is obscured by technical difficulty are marked with [TD].*
3. *Speaker speech is classified based on whether it [Q] asks a question to the Company, [A] provides an answer from the Company, or [M] neither asks nor answers a question.*
4. *This document has been translated by SCRIPTS Asia.*

Disclaimer

SCRIPTS Asia reserves the right to edit or modify, at its sole discretion and at any time, the contents of this document and any related materials, and in such case SCRIPTS Asia shall have no obligation to provide notification of such edits or modifications to any party. This event transcript is based on sources SCRIPTS Asia believes to be reliable, but the accuracy of this transcript is not guaranteed by us and this transcript does not purport to be a complete or error-free statement or summary of the available data. Accordingly, SCRIPTS Asia does not warrant, endorse or guarantee the completeness, accuracy, integrity, or timeliness of the information contained in this event transcript. This event transcript is published solely for information purposes, and is not to be construed as financial or other advice or as an offer to sell or the solicitation of an offer to buy any security in any jurisdiction where such an offer or solicitation would be illegal.

In the public meetings and conference calls upon which SCRIPTS Asia's event transcripts are based, companies may make projections or other forward-looking statements regarding a variety of matters. Such forward-looking statements are based upon current expectations and involve risks and uncertainties. Actual results may differ materially from those stated in any forward-looking statement based on a number of important factors and risks, which are more specifically identified in the applicable company's most recent public securities filings. Although the companies may indicate and believe that the assumptions underlying the forward-looking statements are accurate and reasonable, any of the assumptions could prove inaccurate or incorrect and, therefore, there can be no assurance that the anticipated outcome described in any forward-looking statements will be realized.

THE INFORMATION CONTAINED IN EVENT TRANSCRIPTS IS A TEXTUAL REPRESENTATION OF THE APPLICABLE PUBLIC MEETING OR CONFERENCE CALL. ALTHOUGH SCRIPTS ASIA ENDEAVORS TO PROVIDE ACCURATE TRANSCRIPTIONS, THERE MAY BE MATERIAL ERRORS, OMISSIONS, OR INACCURACIES IN THE TRANSCRIPTIONS. IN NO WAY DOES SCRIPTS ASIA OR THE APPLICABLE COMPANY ASSUME ANY RESPONSIBILITY FOR ANY INVESTMENT OR OTHER DECISIONS MADE BY ANY PARTY BASED UPON ANY EVENT TRANSCRIPT OR OTHER CONTENT PROVIDED BY SCRIPTS ASIA. USERS ARE ADVISED TO REVIEW THE APPLICABLE COMPANY'S PUBLIC SECURITIES FILINGS BEFORE MAKING ANY INVESTMENT OR OTHER DECISIONS. THIS EVENT TRANSCRIPT IS PROVIDED ON AN "AS IS" BASIS. SCRIPTS ASIA DISCLAIMS ANY AND ALL EXPRESS OR IMPLIED WARRANTIES, INCLUDING, BUT NOT LIMITED TO, ANY WARRANTIES OF MERCHANTABILITY OR FITNESS FOR A PARTICULAR PURPOSE OR USE, FREEDOM FROM BUGS, SOFTWARE ERRORS OR DEFECTS, AND ACCURACY, COMPLETENESS, AND NON-INFRINGEMENT.

None of SCRIPTS Asia's content (including event transcript content) or any part thereof may be modified, reproduced or distributed in any form by any means, or stored in a database or retrieval system, without the prior written permission of SCRIPTS Asia. SCRIPTS Asia's content may not be used for any unlawful or unauthorized purposes.

The content of this document may be edited or revised by SCRIPTS Asia at any time without notice.