



Nippon Shinyaku Co., Ltd.

FY2025 Investor Meeting

March 2, 2026

Event Summary

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	Toru Nakai	Representative Director, President
	Takanori Edamitsu	Director, Business Management & Sustainability Division
	Keiichi Kuwano	Director, Research & Development
	Manabu Beppu	Corporate Officer, Head of R&D Planning and Administration Div.
	Hideyasu Takechi	Corporate Officer, Department Manager, Corporate Planning Dept.

Presentation

Takechi: Now, it is time to begin our investor meeting.

As with the R&D briefing, the speakers are Mr. Nakai, Representative Director, President, Mr. Kuwano, Director, Research & Development, and Mr. Edamitsu, Director, Business Management & Sustainability. Thank you.

Then, Mr. Nakai, please start.

Nakai: I am Toru Nakai, President of Nippon Shinyaku. Thank you very much for taking time out of your busy schedule to participate in the investor meeting following the R&D briefing today. Now, we hold the investor meeting as an opportunity for a deeper dialogue with all of you.

First, I will briefly explain our major achievements for the current fiscal year and our plans for launching new products, followed by a question-and-answer session with you. Thank you.

FY2025 R&D Milestones to Date

2025	
Apr	✓ FDA granted Orphan Drug Designation to NS-229 for the treatment of eosinophilic granulomatosis with polyangiitis
Jun	✓ Strategic alliance with Boston Children's Hospital for developing and delivering innovative therapies for rare diseases
Sep	✓ FDA granted Fast Track Designation to NS-229 for the treatment of eosinophilic granulomatosis with polyangiitis ✓ FDA granted Orphan Drug Designation to NS-051/NCNP-04 for the treatment of Duchenne muscular dystrophy ✓ Lilly's antineoplastic agent Jaypirca® 50 mg and 100 mg tablets obtained additional approval as a treatment for relapsed or refractory chronic lymphocytic leukemia resistant or intolerant to other BTK inhibitors
Nov	✓ Japan's MHLW granted Orphan Drug Designation to NS-421/C21 (buloxibutid) for the treatment of idiopathic pulmonary fibrosis ✓ Anti-cancer agent/humanized anti-CD20 monoclonal antibody Gazyva, became available for use in combination with venetoclax for previously untreated chronic lymphocytic leukemia
Dec	✓ ELZONRIS® I.V. Injection received marketing approval in Japan for the treatment of blastic plasmacytoid dendritic cell neoplasm ✓ Johnson & Johnson obtained approval of OPSUMIT® in Japan for the treatment of pediatric patients with pulmonary arterial hypertension ✓ Nippon-Shinyaku and FRONTEO launched co-creation project to evaluate drug discovery seeds

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This slide shows the achievements to date this fiscal year.

Last September, Jaypirca obtained approval for an additional indication for the treatment of relapsed or refractory chronic lymphocytic leukemia that is resistant or intolerant to other BTK inhibitors.

With regard to Gazyva, the combination therapy with venetoclax became available for previously untreated chronic lymphocytic leukemia in November.

As for OPSUMIT, Johnson & Johnson obtained approval for an additional indication for pulmonary arterial hypertension in pediatric patients, and now is preparing for the launch.

This way, we have achieved results that lead to the provision of new treatment options, such as the expansion of indications for several products, and we are making steady progress in our efforts to maximize the value of our products.

In addition, we have received designations, such as Fast Track Designation and Orphan Drug Designation, in the US and Japan for other products under development.

Regarding open innovation, as Mr. Kuwano explained earlier, in June we signed a strategic alliance agreement with Boston Children's Hospital to develop innovative treatments for rare diseases, and in December we launched a co-creation project with FRONTEO to evaluate drug discovery seeds using AI technology.

Overview of the 7th Medium-Term Management Plan

- For Global Growth Beyond the Cliff -

From May 27, 2024
The 7th Five-Year Medium-Term Management Plan, p.19

During the 7th Medium-Term Management Plan, we will promote "three key themes and strengthening five management foundations" to realize Vision for 2035. In each of the Pharmaceuticals and Functional Food segments, we will thoroughly allocate management resources and reduce costs by prioritizing them based on business strategies, and manage the capital efficiency of each segments by ROIC* to secure earnings that exceed the cost of capital.

*ROIC (%) = Operating profit after tax / Invested capital (Non-current assets + Net working capital)

Targets in FY2028	Revenue		Operating profit		
	230 billion yen		30 billion yen		
	EPS 341 yen	ROE 8% or more	ROIC 9% or more		
Three Key Themes	I Fostering growth drivers to replace Uptravi	II Expanding global development	III Continuous pipeline expansion		
Strengthening five management foundations	① Promoting sustainable management for realizing sustainable society	② Speeding up R&D	③ Promoting human capital management that allows each employee to grow and diverse human resources to play an active role	④ Business process reengineering and productivity improvement by promoting digitalization	⑤ Financial strategy for sustainable growth

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The 7th Five-Year Medium-Term Management Plan, which began last fiscal year, promotes three key themes and the strengthening of five management foundations in order to realize the Company's ideal vision for 2035: A global healthcare company from Kyoto creating various types of new ways of life for each person around the world.

One of our three key themes is the continuous pipeline expansion. During the period of this medium-term management plan, we aim to launch an average of at least two new products per fiscal year.

Our Target for New Product Launch

Modified from May 8, 2025
FY2024 Financial Results, p.16

We have been aiming to launch at least 2 new products each fiscal year

1. New indication
2. Newly added
3. Schedule delayed

Period of the 7th Five-Year Medium-Term Management Plan						Period of Next MT Plan	
	FY2024a	FY2025	FY2026	FY2027	FY2028	FY2029	FY2030
Japan	NS-87 (Vyxeos) : high-risk AML	NS-401 (tagraxofusp) : BPDCN	GA101 (Gazyva) : pediatric nephrosis	ZX008 (Fintepla) : CDKL5 gene deficiency	NS-089/NCNP-02 (brogidirsen) : DMD	NS-035 : FCMD ²	NS-050/NCNP-03 : DMD
	LY3527727 (Jaypirca) : r/r MCL	LY3527727 (Jaypirca) : r/r CLL ¹		GA101 (Gazyva) : lupus nephritis			NS-304 (selexipag) : ASO
	NS-304 (Uptravi) : pediatric PAH				GA101 (Gazyva) : SLE without nephropathy ³		
Overseas			CAP-1002 (deramiocel) (U.S.) : DMD cardiomyopathy ³	NS-089/NCNP-02 (brogidirsen) (U.S.) : DMD		NS-050/NCNP-03 (U.S.) : DMD	
						ATSN-101 (U.S.) : LCA1 ³	

Note: NS-051/NCNP-04 (Japan and U.S.) and NS-065/NCNP-01 (Europe and China) are active programs but are currently under ongoing discussions with regulatory authorities. The year of market launch for these products has not yet been determined.

The mucopolysaccharidosis treatments RGX-111 and RGX-121 each received a clinical hold from the FDA in January 2026, and their launch dates are undetermined.

AML: acute myeloid leukemia; r/r MCL: relapsed or refractory mantle cell lymphoma resistant or intolerant to other BTK inhibitors; PAH: pulmonary arterial hypertension; BPDCN: blastic plasmacytoid dendritic cell tumor; r/r CLL: relapsed or refractory chronic lymphocytic leukemia resistant or intolerant to other BTK inhibitors; DMD: Duchenne muscular dystrophy; SEL: systemic lupus erythematosus; FCMD: Fukuyama congenital muscular dystrophy; LCA1: GUCY2D-associated Leber congenital amaurosis; ASO: arteriosclerosis obliterans

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Please move on to slide five.

This slide shows the plan for new product launch by fiscal year.

For CAP-1002 or deramiocel, a cell therapy, Capricor recently submitted the CSR to the FDA and we expect to launch the product in the US during the next fiscal year. NS Pharma, our US subsidiary, continues to prepare for the product launch.

The launch of some products, such as RGX-121 for which REGENXBIO received a Complete Response Letter last month, has been delayed from the original schedule. However, we continue to aim to launch an average of two or more new products per fiscal year, as we announced in May 2024 when we announced our 7th Medium-Term Management Plan.

This concludes my explanation. After this, we will move on to a question-and-answer session. Although time is limited, we hope this will be an opportunity for meaningful discussion with you. Thank you.

Question & Answer

Takechi [M]: Then, we will now go to the question-and-answer session.

Mr. Hashiguchi, please.

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

After you announced your medium-term management plan, the situation for deramiocel has changed repeatedly, and I think that has continued to have no small impact on the company outlook. Once again, given the current situation, I think the timing is about the same compared to what was assumed in the medium-term management plan, but in terms of the data obtained from the trials, indications, and changes in the external environment, are sales likely to go up or be about the same compared to what was assumed in the medium-term management plan? Could you first tell me how you see the overall feeling, especially with regard to the changes in deramiocel?

Nakai [A]: As for how the figures in the medium-term management plan has changed now from what we presented in FY2024, in fact, we believe that the potential of deramiocel or CAP-1002 is higher than that at the announcement of the medium-term management plan.

The reason for this is that the number of patients who would use this product is likely to be higher than at the time of the announcement of the medium-term management plan. Another thing is the price. When we initially presented the medium-term management plan, we provided the figures for maintenance and improvement of upper limb function after conducting a survey with payers and confirming what price they would reimburse. Now, we have learned that if the indication for cardiomyopathy is approved, payers would be willing to reimburse even a slightly higher price.

In terms of volume multiplied by price, the contribution of CAP-1002 to overall sales is considered to be higher at this point than at the time of the announcement of the medium-term management plan.

Hashiguchi [Q]: Is it correct to say that you are now thinking about the price almost exactly the same as you assumed last May when you released the FY2025 forecast?

Nakai [A]: To be specific, we are currently assuming an annual drug cost of USD1 million per patient.

Hashiguchi [Q]: Second, you mentioned earlier that you are simultaneously negotiating for partnering for NS-863, but compared to when you developed Uptravi, what you can do in-house has increased considerably. In other words, I think that continuing to develop independently and partnering after increasing the value of the product may be a viable option.

If you seek a partner, what will you need, what will you expect, and what kind of company will you want to work with?

Also, I would like to know when you think it is likely to be decided at the earliest, given the current state of negotiations.

Nakai [A]: As for what we are expecting for a partner, this is a drug with such potential that we believe it can be a blockbuster drug, so what we seek from our partners is the ability to conduct large-scale global trials, as well as global sales capabilities.

For our part, especially in terms of sales, we only have capabilities for DMD in Japan and the United States. We may gain some capabilities in Europe or China in the future, but from the point of view of such sales capabilities, considering the experience of selexipag, I think that partnering with a company that has sales capabilities will be a very important factor.

Your second question is about timing. In partnering negotiations, I believe the licensor holds the advantage. NS-863 is our in-house developed product, and we stand in that position.

As we have already announced, once deramiocel or CAP-1002 is successfully launched and reaches a stage where growth can be accelerated, we will be able to allocate more resources to R&D as an investment for growth. I believe that we had better work with a partner in a way that increases the value of the product as much as possible, in order to contribute to Nippon Shinyaku's future earnings.

However, in order to have more options available, we are introducing NS-863 to various companies at this time, and are currently obtaining evaluations of the product among such companies. Fortunately, there are companies that have expressed interest. Eventually, we may decide not to collaborate at this stage, but to do the Phase II trial by ourselves. There are many options. However, at the same time, we are also looking for partnering opportunities.

Hashiguchi [Q]: From a global perspective, I feel that it is not too late to start partnering from Phase III, and based on Mr. Nakai's current talk of value enhancement, would it be correct to say that it is more likely that Phase II will be done solely in-house?

Nakai [A]: Well, we are just about to start the Phase II trial, and we are confident that we can handle the operational part. We have not thrown away the possibility that we will have a partner along the way, bearing an appropriate share of the development costs with them. I think we can be flexible in this regard.

Takechi [M]: Mr. Wakao, please.

Wakao [Q]: I am Wakao from JPMorgan Securities.

First, I would like to know if there are any updates on Viltepso in the US.

Nakai [M]: We understand that you are asking about the status of the discussion with the FDA.

Kuwano [A]: As I told you in the last financial briefing, we had not heard back from the FDA by December 23, and when we contacted them after that, they asked us to wait because they are still reviewing the documents. There have been no updates since then.

Wakao [Q]: Did you have a discussion with the FDA at least once, on this matter? Is my understanding correct that you had a meeting with them after the Phase III data was obtained?

The reason I'm asking this question is that Sarepta Therapeutics said they would have a discussion with the FDA after this quarter, and I think they are going to move rather quickly. I am wondering what the difference is, since it is taking you more time. I would like to know if you had any discussions with the FDA in the first place.

Nakai [A]: In October 2024, we submitted a briefing document to the FDA on the premise of having a meeting with the FDA. The content was the results of Study 301, not all of them but some of the results, and our interpretation of them, as well as materials on why it did not achieve the primary endpoint.

In response to that, the FDA told us that they were generally agreeable in concept, but that they would not have a meeting now, because they wanted all the study data, or CSR.

Then, in December, we submitted the complete data, I guess I should say CSR, as well as the protocol for another Study 303. We have been waiting for their response since then, but we have not received anything at all, and the one we finally got was that it is still under review. So we have to wait a little longer.

Wakao [Q]: Your answer matched my understanding, but in the end, why do you think that Sarepta can have a meeting with the FDA earlier than you even though their trial ended after yours? I don't know what form of meeting they will have.

It seems to me that your company may not be very high on the priority list, from the FDA's point of view. Why are you in this situation? Or is there any possibility that the review for your company could go forward after the FDA has had a meeting with Sarepta?

Kuwano [A]: I am not sure about the FDA's intentions. However, while we are explaining how we will conduct the Phase III trial, the media reports that Sarepta will not conduct the Phase III trial, but rather is seeking approval based on the current data. I think they are trying to negotiate for higher hurdles.

So, I think the FDA will first talk with them and then with us, and since both drugs have the same mechanism of action, I think the FDA is preparing the ground so that the response will be the same. This is purely my speculation, though.

Wakao [Q]: If Sarepta and the FDA have a meeting, is there any movement that might occur for your company?

Kuwano [A]: I will have to ask the FDA about this, but for example, if it is possible to get approval based on existing data, we may be able to take a similar approach rather than proceeding with the Phase III study as it is, and if the Phase III study is still needed, Sarepta will have to do that as well.

I don't think it is reasonable to have a completely different response to a similar situation. I think that the FDA is thinking a lot about that while keeping a side eye on it.

However, if they can have a meeting instead of us, I am wondering why they can and wondering if it is true.

Wakao [Q]: Do you think that if Sarepta obtains the traditional approval from the FDA, your company will also be approved without the Phase III trial? If so, why do you think so? Is that because the quality of the data is relatively close or something like that?

Kuwano [A]: No, I don't think the situation in Sarepta will automatically apply to our company.

We have to check how similar their situation is to ours. If negotiations are necessary, we will negotiate with the FDA, and if the Phase III trial is necessary, we will conduct that as scheduled.

Wakao [Q]: At least, if the Phase III trial is not required for Sarepta and they get an approval, is it safe to assume that you may consider not doing the Phase III as a scenario, just in case?

Kuwano [A]: Yes, we think so.

Takechi [M]: Mr. Tanaka, please.

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

In the area of nucleic acids, you mentioned Boston Children's Hospital earlier. Of course, I think your company can do with small molecules and gene therapy, but I think your company's strength from an outside perspective is that you can do with nucleic acids, and I think you are a company that can do it in Japan.

However, DDS has been difficult for many years, and peptides have also become difficult. While you mentioned earlier about transferrin, now, in the area of drug discovery, some people from the outside think that your priority of nucleic acids may be declining. Now, could you please update on that a bit more?

Also, recently, when I look at patents, it seems that you have applied for a patent to change the electric potential. Are you moving ahead with something like that, not just DDS?

Kuwano [A]: You have pointed out that the potential of nucleic acid research is declining. It may seem that way because the parts of the research that are not going well are being revealed, but our in-house stance has not changed at all. We are continuing our basic research.

As you pointed out, we have not been able to find anything definitive for DDS, but we are trying to do so in earnest, albeit belatedly. If we find even one such item that can be used, we would like to apply it to existing arrays and combine them to create new one for development. We would appreciate a little more time.

Tanaka [Q]: I think around December, something like a complex comprising PMO and polyanion appeared in WIPO, so I thought there might have been some movement. However, will it still take a bit of time?

Kuwano [A]: Yes, you are correct.

Tanaka [Q]: Also, with regard to the FDA response earlier, are the people you mentioned earlier who have MD actually working? It would be helpful if you could explain a little more about what kind of person they are.

Kuwano [A]: As for their background, the president explained it earlier. In fact, our negotiations with the FDA have evolved into a more in-depth format than before. Newly hired staff, a new MD, and the existing clinical development team are all collaborating in regulatory affairs. However, as for Viltepso mentioned earlier, even with such a system in place, we still have not received a response. You may think that nothing has changed, but I think that internally our regulatory affairs are becoming quite sophisticated.

Takechi [M]: Mr. Yamaguchi, please.

Yamaguchi [Q]: I am Yamaguchi from Citigroup Global Markets.

Selexipag for ASO, which was mentioned earlier in today's R&D Meeting, is coming out after the expiration of the patent, but of course the patent is still in force. Is my understanding correct that you are aiming for another new indication to make it larger?

Also, is this only in Japan?

Kuwano [A]: Yes, of course, we hope it will be larger, and our development territory is only in Japan.

Yamaguchi [Q]: There have been various drugs for ASO, but since it has grown so large in Japan, it is being developed because you think it can be used in that area, right?

Kuwano [A]: Yes, you're right. We have been working on this for a long time, even before working on the indication for pulmonary hypertension, but it is a very difficult area for clinical evaluation, and we have finally found a way through our own struggles. We have done the Phase II studies multiple times, and in that process, we have already gotten to the point where we can do the Phase III study well, so we are hoping to somehow do one more study for reproducibility and finish it.

Yamaguchi [Q]: Also, there was a full disclosure today about NS-863. I believe that you have accumulated a lot in this area, as you were originally a master of this field. I am sure you are well aware of the global situation, and this is the first time that indications have been revealed. Are you confident that this will work?

Kuwano [A]: Actually, I've been involved with NS-863 since the exploratory research phase and have also participated in the design process. Since it's a development project we've nurtured together from the very beginning, I feel a special connection to it. As I mentioned earlier, we believe we have a PoC for clinical effectiveness, so I think we have a good chance of winning. It is our specialty and we want to commercialize it successfully.

Yamaguchi [Q]: Also, just to confirm something basic for deramiocel, you mentioned earlier that you submitted the CSR, but I am just wondering if the PDUFA has been decided or not yet?

Nakai [A]: Some day in February, Capricor submitted the CSR to the FDA. It will take a few weeks for the FDA to contact Capricor with the acceptance of the CSR, and along with the acceptance from the FDA, PDUFA will probably be informed of us. That's where we are right now and the ball is in the FDA's court right now.

Yamaguchi [Q]: You mentioned before that you think that would come in February, but now that will come by the end of March, because it takes a few weeks. So, after the PDUFA is determined, it will be subject to press release in many ways.

Nakai [A]: Well, if the PDUFA is notified by the FDA in March, their review clock will start from there, so we are now assuming that the PDUFA date will be September this year, since it is six months from March.

Yamaguchi [Q]: Also, of course, NS-863 is not yet included in the launch target of this medium-term management plan because you will finish Phase II in 2028, but if you extend the product launch schedule a little further, will that appear in this schedule? You cannot finish that by 2030, but can you do by 2031 or maybe 2032?

Nakai [A]: Well, we have not decided on how many years the next medium-term management plan will cover, but if we assume it will cover five years, NS-863 will be launched during the next medium-term management plan, and we would like to develop it that way.

Takechi [M]: Mr. Lee, please.

Lee [Q]: I am Lee from Morgan Stanley MUFG Securities.

I would like to ask you about CAP-1002. What is your current view on the acquisition of Capricor Therapeutics?

For example, Mr. Nakai has mentioned that the corporate cultures of both companies have always been a good fit. If we look at the data from the Phase III study at the end of the last year, it seems that the potential is huge, so I think there could be a significant return even from the current perspective. On the other hand, you explained about the JPY100 billion capital allocation in this medium-term management plan before. I think it is a difficult balance to strike, but given the significant product potential of CAP-1002, there appears to be room for investment. What do you think?

Nakai [A]: I can't talk about specifics here, but we are constantly discussing with Capricor how to maximize the global product value of deramiocel, including the adding new indications and the manufacturing capacities.

Therefore, I believe that the appropriate form of alliance, not limited to an acquisition, will emerge after confirming how we can expand this product and what roles both companies can play in it.

Lee [Q]: As a follow-up question, Capricor has been saying that they will focus the US approval first. What about Europe? I believe your company still holds the option right to sell deramiocel in Europe. Do you plan to establish a European company from the beginning? Or will M&A and other initiatives be one option? Of course, I understand very well that you will do in the US first, but I would like to know the situation for the next three or four years, please.

Nakai [A]: At first, we both confirmed that if we successfully launch deramiocel in the US, we would like to move forward with expansion into Japan and Europe, but again, there is also talk of drug pricing or a Most-Favored-Nation policy, which is a risk unique to the US.

We are now faced with the issue of how the European product launch will affect the price in the US, and both companies need to carefully examine the impact of this on the US price. We are checking these points with both companies, being careful not to rush inappropriately, which could undermine the global value of deramiocel.

Our status in Europe is that we have the exclusivity to negotiate the European rights of deramiocel. Capricor is currently in a position where only we Nippon Shinyaku can negotiate that matter.

Lee [Q]: Lastly, I'm very sorry to be technical, but now that you have adopted IFRS, are you thinking of introducing, for example, a core OP like other companies?

For example, if this CAP-1002 is approved, then depreciation and other expenses will probably be recorded in the current PL, so I am thinking that it would be very easy to understand if there is a core OP in a way that excludes those expenses. Please let me know what you think about this.

Nakai [A]: I have been discussing with Edamitsu about what kind of policy should be adopted in this area as well. If there is a time when we can announce the introduction of core operating profit, for example, we would like to do so at the appropriate time, but we have not yet decided on it.

Lee [Q]: Just to confirm, am I correct in understanding that amortization is posted as costs, not as SG&A?

Edamitsu [A]: You are correct.

Takechi [M]: Mr. Wada, please.

Wada [Q]: I am Wada from SMBC Nikko Securities.

You told in answer to Mr. Hashiguchi's question about CAP-1002 that the range of eligible patients is expanding, but when I look at the data, the difference between HOPE-2 and HOPE-3 seems to have become smaller a little, although the difference is significant enough. I would like to ask you what you think of the reasons for this background.

Also, based on that, how is the number of indicated patients likely to affect this efficacy? Can you tell me what you think now?

Kuwano [A]: Looking at the numbers alone, it may appear as you say, but HOPE-2 is a small study with only about 20 cases, and HOPE-3 is more than five times that number. I think the reality has become clear.

However, whatever the absolute value of efficacy there, I think it is all about being effective with a firm and significant difference, so I think that is not a problem.

Wada [Q]: Also, as for PUL2.0, we have the data from the paper, and if we compare with Viltepso, it looks like Viltepso is stopping the progression of the disease, but it seems to be progressing with CAP-1002. What are your thoughts on this difference?

What I have as a hypothesis is that the disease appears to be progressing at the time of the CAP-1002 trial because the severity of the disease is different. I wonder if you could give me your thoughts and background on this.

Kuwano [A]: Yes, as you say, different patient backgrounds may have an influence, and considering that the patients also have cardiomyopathy, I think you are right that it is difficult for the drug to work as well as Viltipso.

Wada [Q]: Finally, I would like to ask about an advisory committee. As for CAP-1002, do you think basically there will be an advisory committee before the PDUFA?

Nakai [A]: As for whether there will be an advisory committee or not, after the review clock starts moving forward again, we will receive suggestions from the FDA at the mid-cycle meeting, late-cycle meeting, or at such other time. So we do not know if there is or is not in a situation where the review clock has not moved yet.

Takechi [M]: Mr. Sakai, please.

Sakai [Q]: I am Sakai from UBS Securities.

As for the partnership with Boston Children's Hospital, which was introduced this time, does the fact that it was introduced in this way after six months has passed indicate there is a concrete project moving forward in the future? What kind of scheme would your company use for this? I think it would be in the form of an open call, so would your company cash out so to speak and invite interested parties?

Or, of course, there are already patients in the hospital, so I don't know if it is correct to call it biopsies, but is there a possibility to conduct some kind of research using such biopsies in the future? Is it possible for you to introduce us to this?

Kuwano [A]: Actually, I think we will be able to report soon how we will do it. We are in the final stages of finalizing the contract, so I can't go into details, but we have actually already done the public solicitation as you pointed out and have already narrowed cases down. Specifically, we are now in the process of finalizing the contract to collaborate with the doctors there in the future.

Naturally, leveraging the characteristics of BCH as a medical institution, we expect to be able to use patient specimens for research, as you have indicated, and if the research progresses, we would like to consider further and new developments afterwards.

Sakai [Q]: So then, I don't know if it's good or bad to call them partners, but is it correct to say that they are doctors who work at the hospital?

Kuwano [A]: Yes, we will be working with doctors who have a kind of laboratory.

Takechi [M]: Mr. Yamakita, please.

Yamakita [Q]: I am Yamakita from Jefferies.

Let me ask one question about costs. Of course, if CAP-1002 or deramiocel and other products sell well, I understand that there is no need to worry about the cost level at all, but we must also consider the worst-case scenario.

It seems that several Phase II trials will start in the next fiscal year, and some of the commercial functions of deramiocel have already been added and will be added further in the next fiscal year. Accordingly, SG&A expenses and R&D expenses combined will total about JPY90 billion. And I am concerned that this cannot be reduced much. I would like to know once again your thoughts on the costs that will be incurred.

Also, regarding R&D expenses, since Mr. Kuwano was newly appointed, I wonder if there has been any change in the stance here in terms of the selection of pipeline items. Could I ask you to explain the costs from this perspective as well?

Nakai [A]: First of all, we are beginning to consider the budget for the next fiscal year under the assumption that CAP-1002 in particular will be successfully approved around September this year. As a rough image, if we proceed according to the assumptions we have in mind, we are returning to a growth trend in terms of profits.

Under such circumstances, where we start the development of NS-863, NS-035, and NS-304 for ASO, as introduced today, even if we have to conduct another Phase III trial for Viltepsa, we feel that we can grow on a profit basis as well.

With regard to your question about a more pessimistic situation, I am aware that when CAP-1002 does not sell well or its launch is postponed, it is necessary to take steps to curb the SG&A expenses in the US.

Also, as for the development of the products that require large R&D expenses, such as NS-863, for which we received a question earlier about out-licensing, we believe that we can control the cost of such assets by working with partners if it is quite difficult to incur such expenses internally.

As for the question of whether our priority has changed since Mr. Kuwano took over as the head of R&D, we may have to consider what we are going to do about the development of exon skipping drugs, such as the 44-skipping drug NS-089, depending on the situation of Avidity Biosciences, and whether NS-051, which is currently on clinical hold by the FDA, is competitive in the 51-skipping area. We will prioritize the development of each product, taking into consideration the competitive relationship and the status of negotiations with the authorities, and will make a proper decision on whether or not each product is competitive to secure profits.

Yamakita [M]: I now understand that you will still be able to make a profit.

Takechi [M]: Mr. Muraoka, please.

Muraoka [Q]: I am Muraoka from Morgan Stanley MUFG Securities. Thank you.

As for DMD's strategy in Japan, recently, other companies have acquired DMD drugs in Japan, and they are talking as if they are marketable, but I am not sure about that.

If there is a DMD drug in Japan with a mode of action that does not compete with exon skipping, they would first contact you. But other companies ended up acquiring rights instead of you. Didn't you raise your hand because of the competitive relationship? Or weren't you interested because you didn't create any synergy at all? I'm asking this on the assumption that you have probably been contacted about those opportunities, but is there anything you can comment on?

Nakai [A]: First, please understand that it is not easy to comment on undisclosed information from other companies.

In the DMD disease area and in the Japanese market, there are probably about two products that have been contracted with other companies. I have the impression that the contracts are based on the premise that there are huge sales potentials. I think it is still a business decision for each company to decide how to think about this.

As far as we are concerned, we need to consider the area, country, modality, and stage when acquiring assets, as there are partners we have to consider. For example, we are prioritizing the acquisition of assets in the US, and in Japan, we do not need to be so aggressive for DMD in acquiring assets.

If we are the only company in the DMD product market, we will have to be concerned about antitrust regulations from the standpoint of competition. I would like you to understand that there is also a legal aspect and we cannot create an environment where we as a single company monopolizes the market.

Muraoka [Q]: Also, on the subject of deramiocel, in your comment earlier about how you are in close communication with Capricor, you said that you communicate with them in various ways, including manufacturing. However, there is something we can't see from the outside, and last year, suddenly the CMC-related issue involving the CRL came up. It has been already cleared up, but honestly, it is a black box and hard to see from the outside.

Am I correct in assuming that this CMC issue has already been resolved and there is no need to worry about it?

Nakai [A]: Yes, as far as we can see, the authorities actually came to inspect their manufacturing facility in San Diego and gave them some homework assignments. Then, Capricor addressed that issue and they directly reported to us that the FDA had been satisfied with their action. Also, our quality assurance team has also visited their San Diego manufacturing facility to communicate with their relevant departments.

In order to eliminate the risk of non-approval as much as possible, we are of course checking the quality and CMC of each product as we monitor the status of the review. I would like you to understand that we appropriately deal with that.

Takechi [M]: Mr. Wakao, please.

Wakao [Q]: I would like to confirm one thing. I believe Capricor will be presenting details at the conference in March, and I am wondering if you could tell me anything about HOPE-3 that I should pay attention to. The top line data has been disclosed, so we are only getting the detailed figures and such, but I would like to know if there is anything that would help me see CAP-1002 or deramiocel further up, or in some cases, lower.

Kuwano [A]: We have not looked at the exact data to be released in the presentation, but I think the essence is what is presented in their top line data, and I am sure that they will report various data that will reinforce that in more detail. In any case, I hope you will pay attention.

Takechi [M]: Since there seems to be no one else with questions, I will conclude the question-and-answer session.

This concludes the Investor Meeting. Thank you very much for joining us today over such a long period of time.

[END]