Sumitomo Pharma

Sumitomo Pharma Co., Ltd.

R&D Meeting

February 17, 2026

Event Summary

[Company Name]	Sumitomo Pharma Co., Ltd.
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[Event Name]	R&D Meeting
[Fiscal Period]	
[Date]	February , 2026
[Number of Pages]	53
[Time]	14:00 – 15:46 (Total: 106 minutes, Presentation: 50 minutes, Q&A: 56 minutes)
[Venue]	Webcast and in-person at the Osaka headquarters
[Venue Size]	
[Participants]	
[Number of Speakers]	5

Toru Kimura	Representative Director, President and CEO
Motoyuki Sakai	Representative Director, Executive Vice President Global Corporate Strategy; Global Finance Administration External Affairs; Corporate Governance; IT Management & Data Analytics
Yumi Sato	Managing Executive Officer, Research and Development Division, Senior Vice President, Head of Research and Development Division, Chief Development Officer, Sumitomo Pharma America, Inc.
Masashi Murata	Global Oncology Strategy Lead, Lead Senior Vice President, Sumitomo Pharma America, Inc.
Koichi Kino	Vice President, Head of Corporate Governance

[Analyst Names]	Kazuaki Hashiguchi	Daiwa Securities
	Seiji Wakao	JPMorgan Securities
	Shinichiro Muraoka	Morgan Stanley MUFG Securities
	Hiroshi Wada	SMBC Nikko Securities

Presentation

Kino: We will now begin the R&D meeting of Sumitomo Pharma Co., Ltd. Thank you very much for joining us today.

I am Kino, Head of Corporate Governance, and I will be your moderator today.

Today's session will be conducted in a hybrid format with a face-to-face meeting at the Osaka headquarters and a Zoom webinar. Analysts and investors are invited to participate online, and media are invited to participate either in person or online. We will also provide a brief opportunity of social gathering for the media.

To ensure smooth proceedings, please change the participant information displayed on your Zoom screen to your company name and your name.

First, I will explain our R&D progress, basic policy, and two major oncology products using the presentation material available on our website, followed by a question-and-answer session. The end time is scheduled for 15:40.

In attendance today are Mr. Kimura, Representative Director, President and CEO, Mr. Sakai, Representative Director, Executive Vice President, Ms. Sato, Managing Executive Officer, and Mr. Murata, Global Strategy Oncology Lead.

First, Mr. Kimura will say a few words.

Kimura: Thank you very much for attending our R&D presentation today. I would like to say a few words at the opening of the meeting.

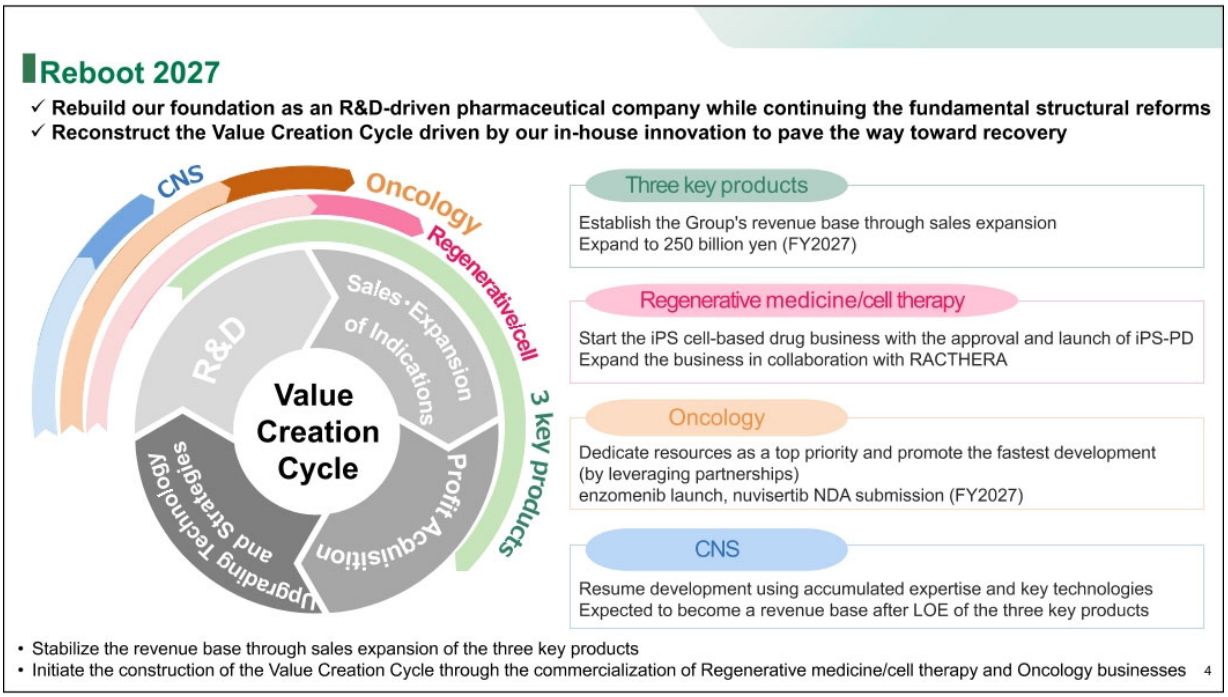
Last Friday the 13th, the agenda for the Regenerative Medicine Products and Biologics Technology Subcommittee of the Pharmaceutical Affairs Council of the Ministry of Health, Labor and Welfare, scheduled to meet on February 19, was announced. Non-autologous iPS cell-derived dopamine neural progenitor cells, for which we have applied for approval, were also on the agenda. Since the product name AMCHEPRY is to approved by the subcommittee, we had not disclosed it before, but on the 13th, we disclosed that this is our product.

The subcommittee will discuss the approval of AMCHEPRY, and then a final decision will be made by the Minister of Health, Labor and Welfare at a later date. We will keep you informed of any developments in this regard.

Kino: Now, let's move on to today's presentation.

First, Sato will explain our R&D progress and basic policy. Ms. Sato, please proceed.

Sato: Thank you very much. My name is Sato, and I am in charge of R&D. Thank you.



See page four.

Last May we published Reboot 2027. With this, we have declared that, in parallel with the continuation of fundamental structural reforms, we will work to rebuild our foundation as an R&D-oriented firm and pave the way for revival by rebuilding a value creation cycle based on our own innovations.

Here is the status of achievement of R&D milestones aimed for FY2025. Murata will explain the progress in the oncology area later.

In the area of regenerative medicine and cell therapy, As Kimura just mentioned, the Regenerative Medicine and Cell Technology Subcommittee is scheduled to deliberate on our product this week.

In the area of infectious diseases, we are currently conducting Phase I trials of a universal influenza vaccine. We reported last fall that an interim analysis confirmed that the new adjuvant was well tolerated and that antibody titers increased, suggesting the efficacy of the vaccine. We are continuing to analyze cross-reactivity and viral activity to confirm universality.

Development Pipeline (as of January 30, 2026)

Area	Generic name/Product code	Mechanism of action, etc.	Planned indication(s)	Development stage
Psychiatry & Neurology	DSP-0038	Serotonin 5-HT_{2A} receptor antagonist and serotonin 5-HT_{1A} receptor agonist	Alzheimer's disease psychosis	Phase 1
	DSP-0187*	Selective orexin 2 receptor agonist	Narcolepsy	Phase 1
	DSP-3456	Metabotropic glutamate receptor 2/3 negative allosteric modulator (mGluR2/3 NAM)	Treatment resistant depression	Phase 1
	DSP-0378	Gamma-aminobutyric acid $(GABA)_A$ receptor positive allosteric modulator	Progressive Myoclonic Epilepsy Developmental Epileptic Encephalopathy	Phase 1
	DSP-2342	Serotonin 5-HT_{2A} and 5-HT_7 receptor antagonist	To be determined	Phase 1
	CT1-DAP001/DSP-1083 (Japan)	Allogeneic iPS [induced pluripotent stem] cell-derived dopaminergic neural progenitor cells	Parkinson's disease/Investigator-initiated study	MAA submitted in August 2025
	CT1-DAP001/DSP-1083 (U.S.)	Allogeneic iPS cell-derived dopaminergic neural progenitor cells	Parkinson's disease/Investigator-initiated study, Company- sponsored clinical study	Phase 1/2
	HLCR011(Japan)	Allogeneic iPS cell-derived retinal pigment epithelial cells	Retinal pigment epithelium tear	Phase 1/2
	DSP-3077(U.S.)	Allogeneic iPS cell-derived retinal sheet	Retinitis pigmentosa	Phase 1/2
Oncology	Enzomenib/DSP-5336	Selective menin inhibitor	Acute leukemia	Phase 2
	Nuvisertib/TP-3654	PIM1 kinase inhibitor	Myelofibrosis	Phase 1/2
	SMP-3124	CHK1 inhibitor	Solid tumors	Phase 1/2
	DSP-0390	EBP inhibitor	Glioblastoma	Phase 1
Others	KSP-1007	β-lactamase inhibitor	Complicated urinary tract and intraabdominal infections, Hospital-acquired bacterial pneumonia	Phase 1
	fH1/DSP-0546LP	Split, Adjuvanted vaccine	Influenza virus prophylaxis	Phase 1

*Development rights: Japan, China, and certain Asian countries

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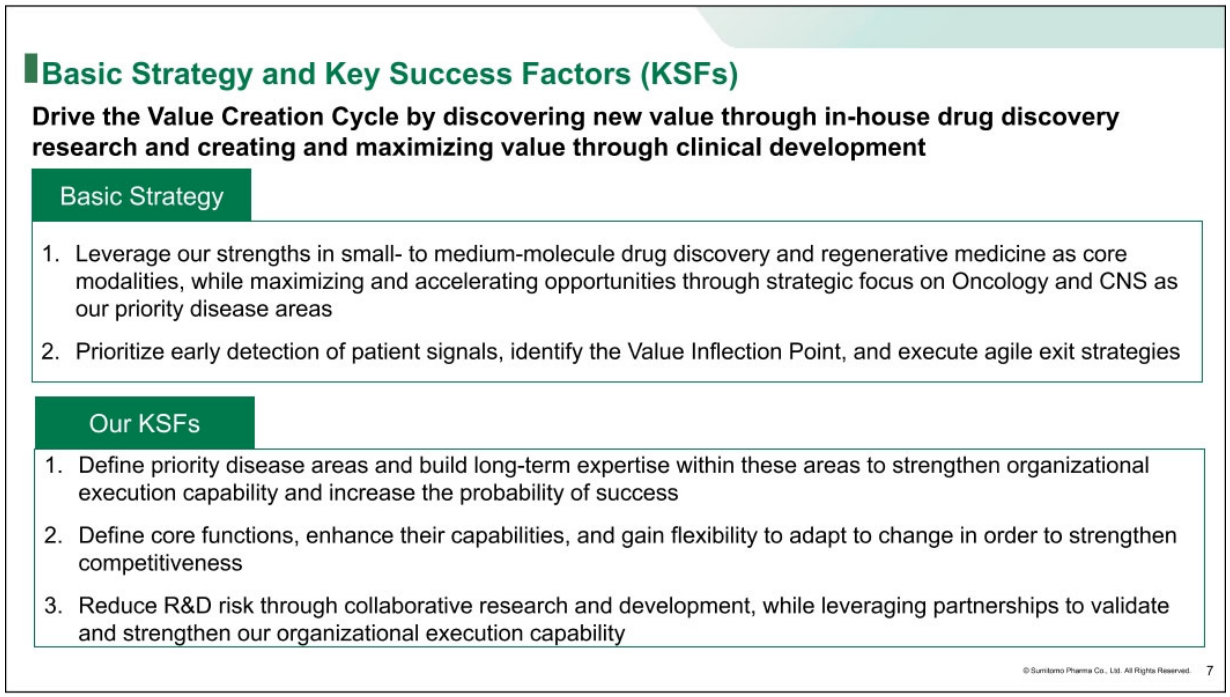
Here is our pipeline list.

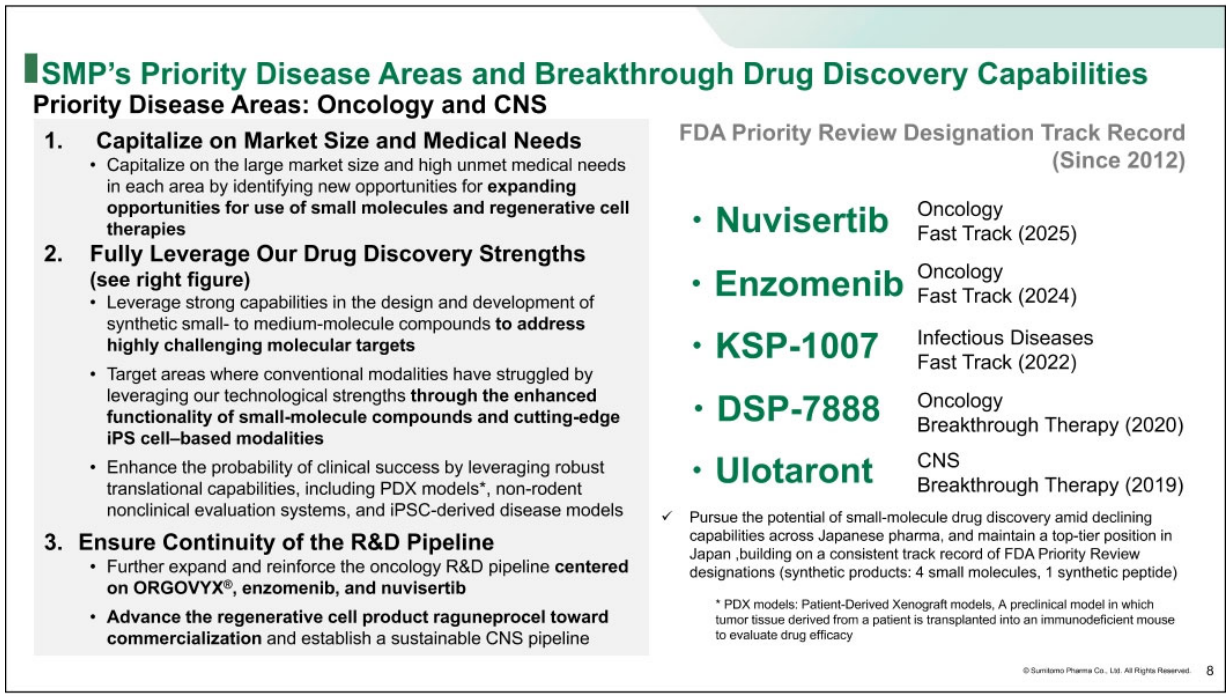
We have shown you about regenerative medicine for Parkinson's disease. You are aware that we are conducting a confirmatory and application study on enzomenib. Other than that, there are no late-stage development items, but rather a pipeline of early-stage development products, and how to move this forward is an urgent and important issue for our company.



We describe our basic R&D strategy here.

We would like to turn the value creation cycle around by discovering new value through in-house drug discovery research, creating value through clinical development, and maximizing value.

Our basic strategy is to maximize and accelerate opportunities by selecting and focusing on our core disease areas of oncology and CNS, with our strengths in small- to mid-molecular drugs and iPS-derived cells as our modality axis. We will implement an agile exit strategy with a strong emphasis on acquiring patient signals early, with a firm focus on identifying the value tipping point.

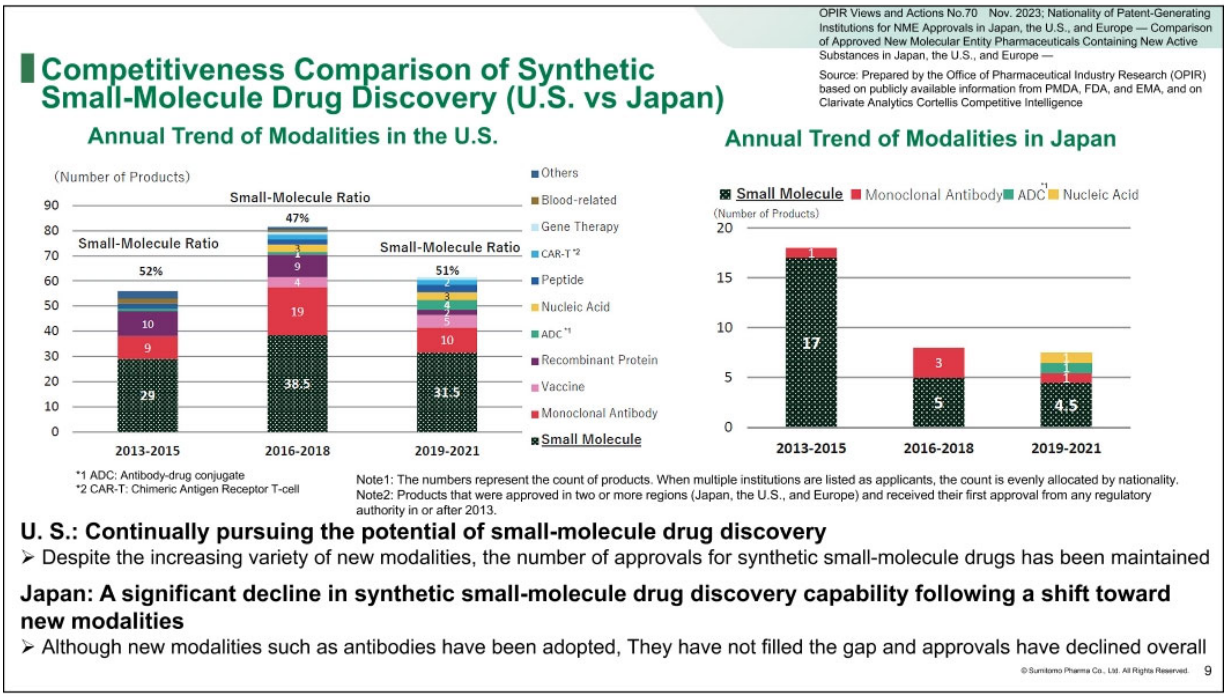


However, I just mentioned that the core disease areas are oncology and CNS. We recognize that both of these areas have a large number of patients, that unmet medical needs continue to be high, and that the use of small molecule drugs and regenerative cells is advancing in these areas.

We recognize our strength in the design, synthesis, and development of small to mid-molecular drugs and our ability to handle highly challenging target molecules. On the right side, we have shown the results of obtaining priority review designation from the FDA since 2012. 5 items are listed here, items for which we have acquired the designation. They are all low to medium molecular weight. We recognize that this number is one of the highest among domestic pharmaceutical companies.

In oncology and CNS, there are many targets that have been difficult to target with traditional modalities. We believe that we will be able to take advantage of our technological capabilities through the functionalization of small molecule drugs and the use of iPS cells, a cutting-edge modality. In addition, as we have shown here, we have several substantial translational technologies that we believe will allow us to proceed with development with a high degree of certainty of clinical success.

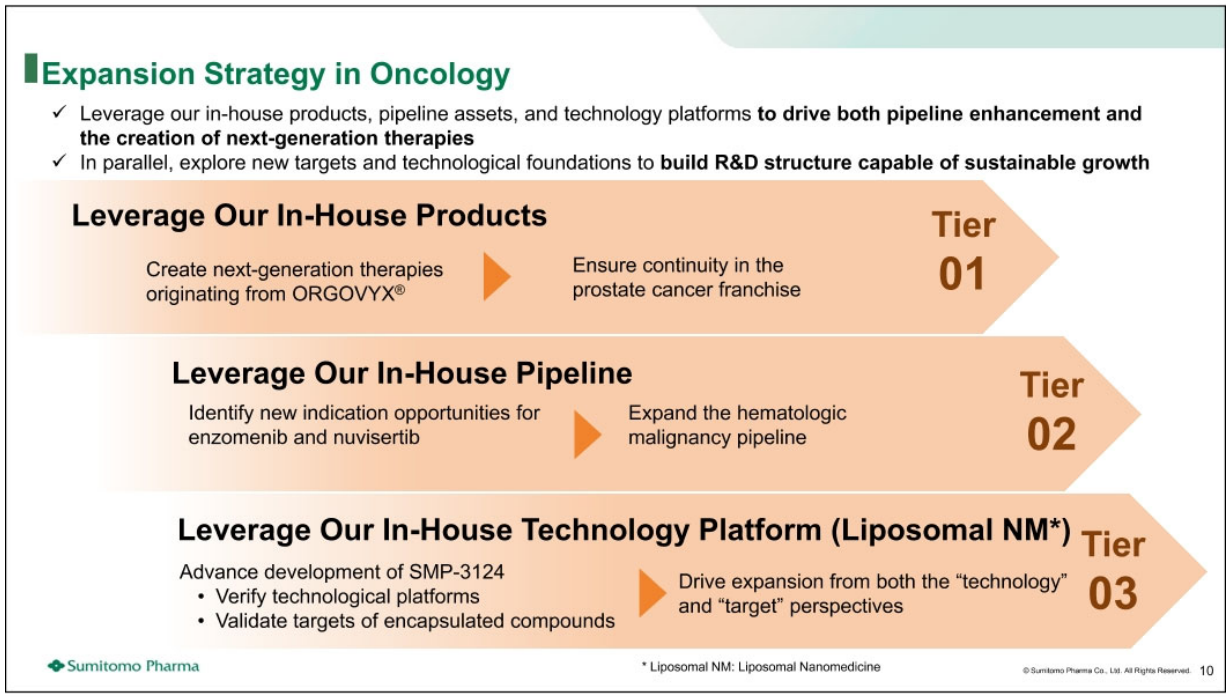
We will further expand and augment our oncology R&D pipeline with existing pipelines such as ORGOVYX, enzomenib, and nuvisertib. We would then like to establish a CNS research pipeline with continuity while monitoring the progress of development of raguneprocel.



Here is some information from the Policy Research Institute's newsletter about the competitiveness of synthetic small molecule drug discovery. We chose Japan and the US as the countries of creation. The vertical axis shows the number of approved products globally, and the breakdown by modality is shown by time period.

What this shows is that Japan's synthetic small molecule drug discovery capabilities are declining as a result of shifting its focus to new modalities. Although new modalities such as antibodies have been introduced, the current number of approved drugs does not significantly compensate for this.

Meanwhile, synthetic small molecules, shown in black, continue to be approved in the United States. The number of approved synthetic small-molecule drugs has been maintained while the number of new modalities has increased. We recognize that the information supports the possibility that synthetic low-molecular-weight drugs may continue.



Here you see our expansion strategy in the field of oncology.

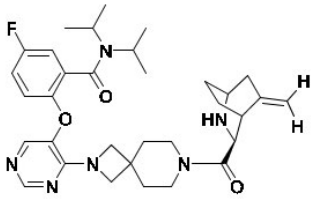
Tier one is "leverage our in-house products." We are working to ensure the continuity in the field of prostate cancer franchise as a next-generation drug creation starting from ORGOVYX.

Tier two is "leverage our in-house pipeline." We will continue to expand our hematopoietic malignancy pipeline by utilizing the knowledge and information obtained through the promotion of enzomenib and nuvisertib development and indication expansion activities.

Tier three is "leverage our in-house technology platform (here, liposomal nanomedicine technology)." We intend to build a pipeline utilizing this technology while continuing to validate the technical basis of the SMP-3124 technology and the targets of the encapsulated compounds.

Enzomenib（DSP-5336）

Leveraged co-creation with academia as a starting point, combining our precise compound-design and synthesis capabilities with forward-looking competitive profile design to generate well-differentiated assets



- A small-molecule compound **with inhibitory protein-protein binding activity for a highly challenging target**
- Compound design with a complex structure **that breaks from conventional norms for orally available small molecules**
- **A robust patent portfolio** amid intense competition

Discovery of drug targets driven by co-creation with academia

- ✓ Engaged Dr. Akihiko Yokoyama, an early pioneer in menin science, as Principal Investigator through the DSK Project*1

Compound design capabilities and MD simulation *2

- ✓ Discovered new target binding sites and designed compounds with efficient binding properties
- ✓ Organic synthesis capability enabled the practical production of industrially scalable compounds

Profile design anticipated intense competitive environments

- ✓ Prioritized strong pharmacological activity while minimizing cardiotoxicity in light of competitive pressures
- ✓ Developed biomarkers to enable patient stratification and clinical efficacy prediction

*1 DSK Project: A cancer drug discovery project under an industry–academia collaboration between Kyoto University and Sumitomo Pharma, from fiscal year 2011 (start of the first term) to fiscal year 2020 (end of the second term)
*2 MD simulation: Molecular Dynamics simulation A computational method that reproduces the movements of compounds and proteins at the atomic level and evaluates binding modes and stability

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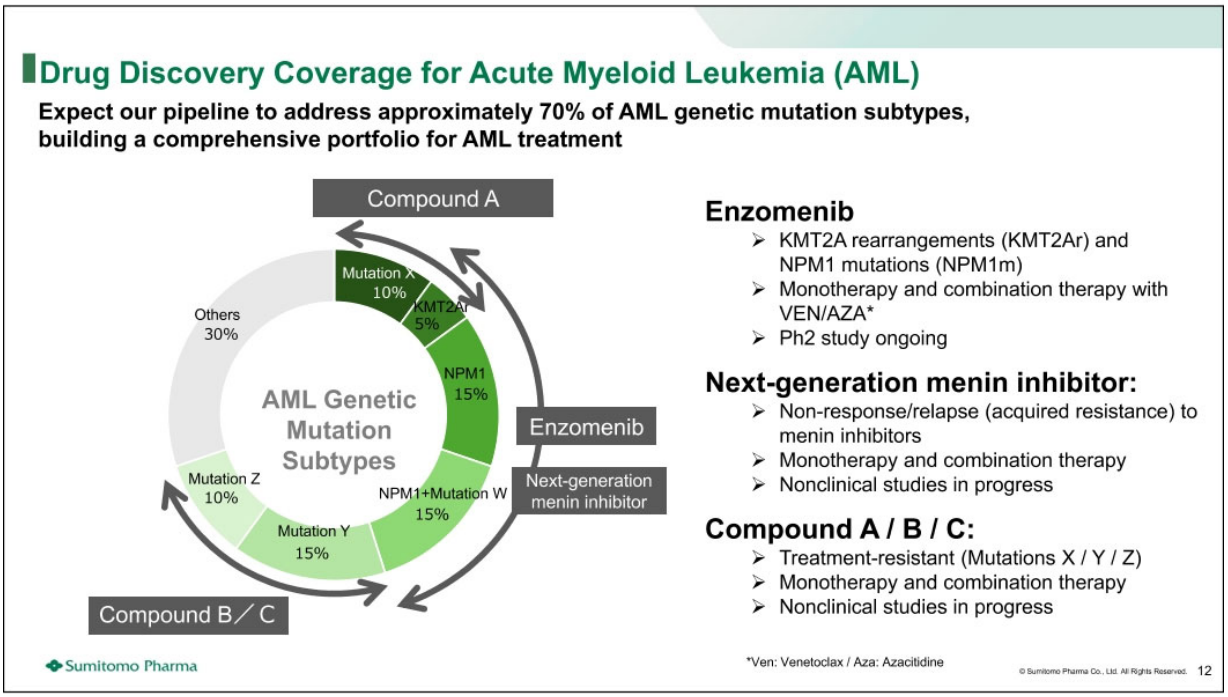
Here is a brief history of the creation of enzomenib.

We have discovered drug targets through co-creation with academia. enzomenib was created by combining the discovery of new target binding sites, the ability to design compounds that bind efficiently, and the ability of organic synthesis to actually create compounds that can be industrialized.

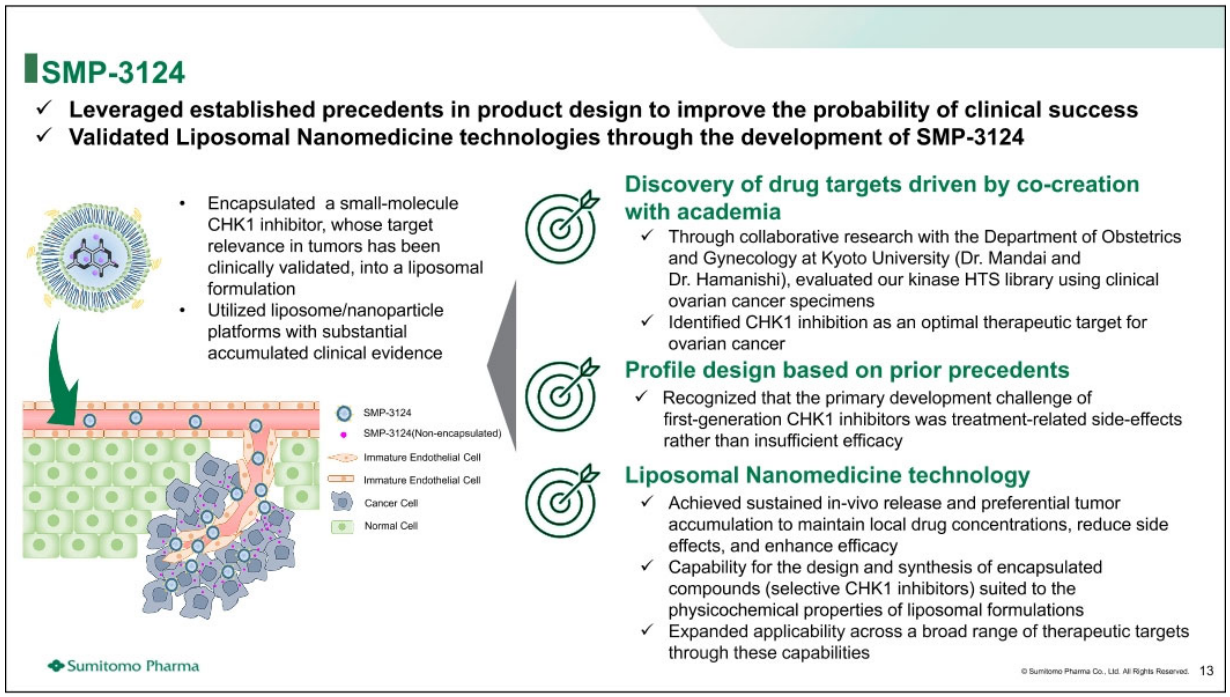
In addition, in anticipation of fierce competition, we have created this compound by promoting drug discovery activities that emphasize high pharmacological efficacy and avoidance of cardiotoxicity.



This is our drug discovery strategy for acute myeloid leukemia (AML).

We are currently developing enzomenib for KMT2A reconstitution and NPM1 mutation.

Next-generation menin inhibitors are intended for patients who do not respond to existing menin inhibitors or who relapse. We are also working on the discovery of another compound that can cover a different mutation than enzomenib. Ultimately, we hope to build a pipeline group that will cover about 70% of the total.

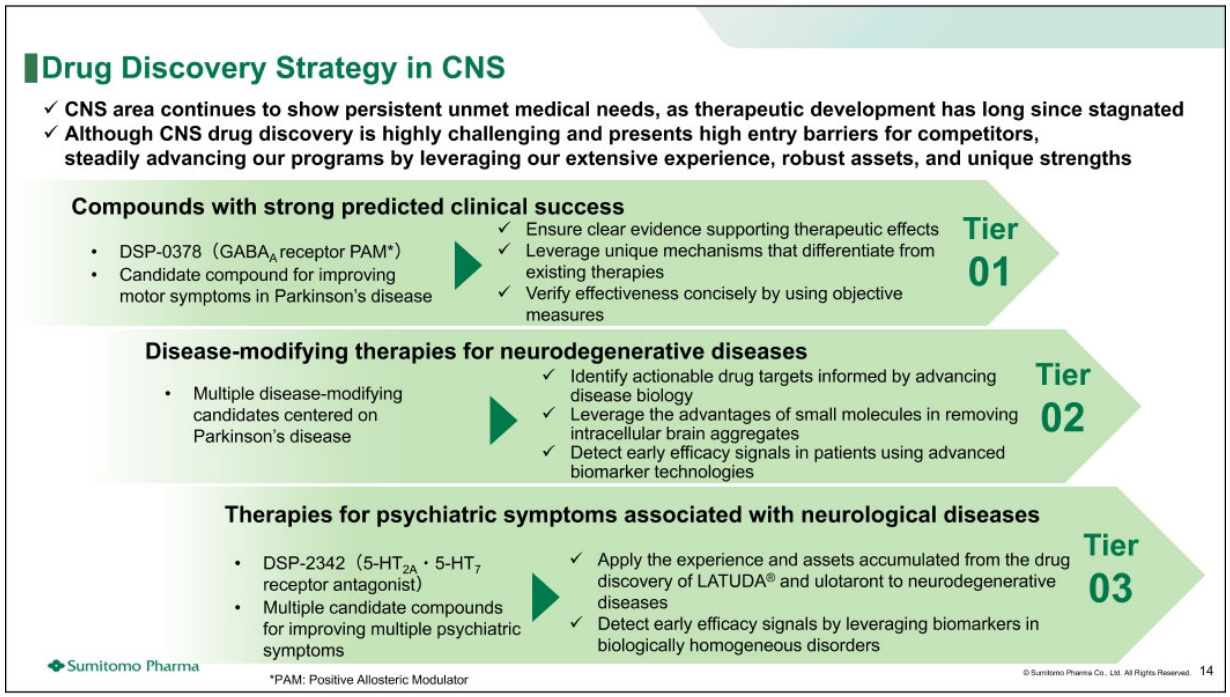


SMP-3124.

In this regard, we discovered a drug target through co-creation with academia, and found that CHK1 inhibitors are the optimal target for ovarian cancer.

Development of existing prior CHK1 inhibitors has stalled. Focusing on the fact that the lack of efficacy is not the reason for this, but rather the narrow safety zone, we have developed the concept of using liposomal nanomedicine technology to ensure a safe zone through sustained release and drug accumulation effects on cancer tissue.

In addition, we found this compound based on the concept of designing and synthesizing an encapsulated compound suitable for the physical properties of liposome formulations. We are currently confirming this concept in a Phase I study.



I would like to move on to the drug discovery strategy in the CNS area.

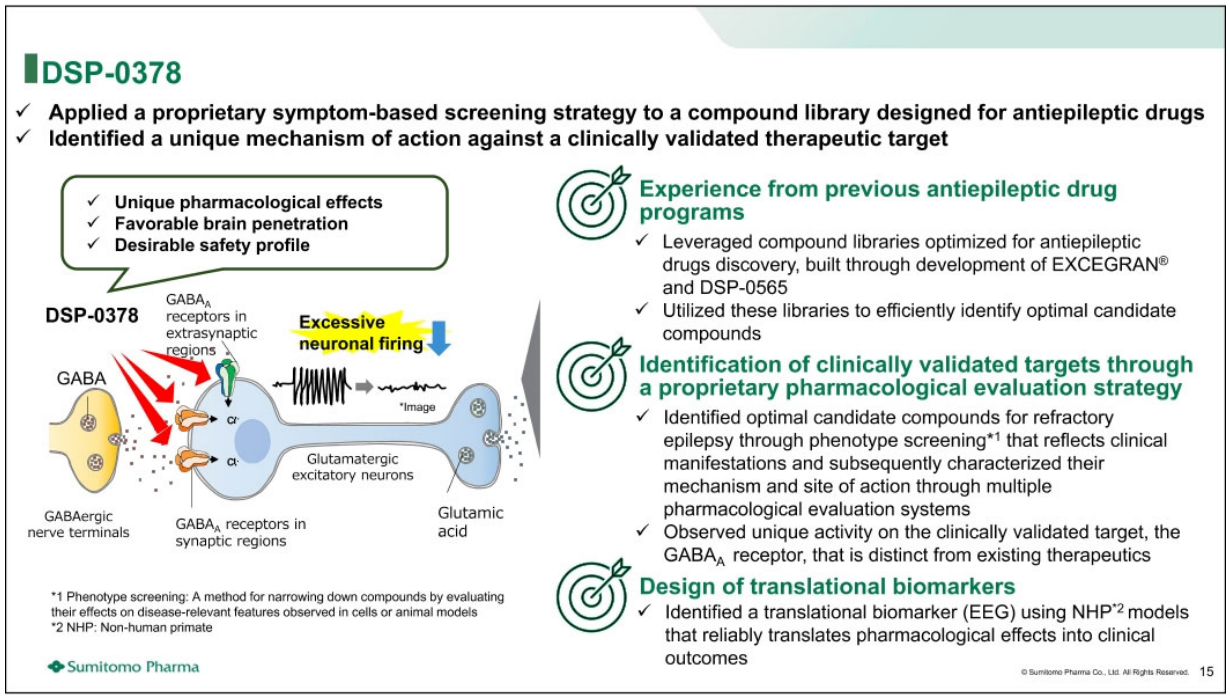
In the CNS area, drug development leading to treatment is stagnant, and medical needs remains over the long term. We recognize the high degree of difficulty in drug discovery and the high barriers to entry for other companies. We would like to steadily develop drug discovery by utilizing our long and abundant experience, as well as the assets and strengths we possess.

tier one is a group of products that are expected to have a high probability of clinical success. These are compounds that have unique actions that are clearly supported by their therapeutic effects and that differentiate them from existing drugs. We would like to proceed to confirm the effectiveness concisely by utilizing objective indicators. We will soon begin clinical development of DSP-0378, a drug for rare epilepsy, and a drug candidate for improving motor symptoms of Parkinson's disease.

tier two is for disease-modifying drugs for neurodegenerative diseases. This is an area where pathophysiology is being elucidated, and it is becoming possible to identify drug targets to be addressed. We are in the process of creating several disease-modifying drug candidates, mainly for Parkinson's disease.

tier three is a group of drugs for the treatment of psychiatric symptoms associated with neurological disorders. The experience and assets accumulated in LATUDA and ulotaront will be used for neurodegenerative diseases. We would like to detect efficacy signals early by utilizing biomarkers in diseases with homogeneous backgrounds.

These are all at very young development stages, and our urgent task and goal is to focus on early clinical development and generate late-stage development items.

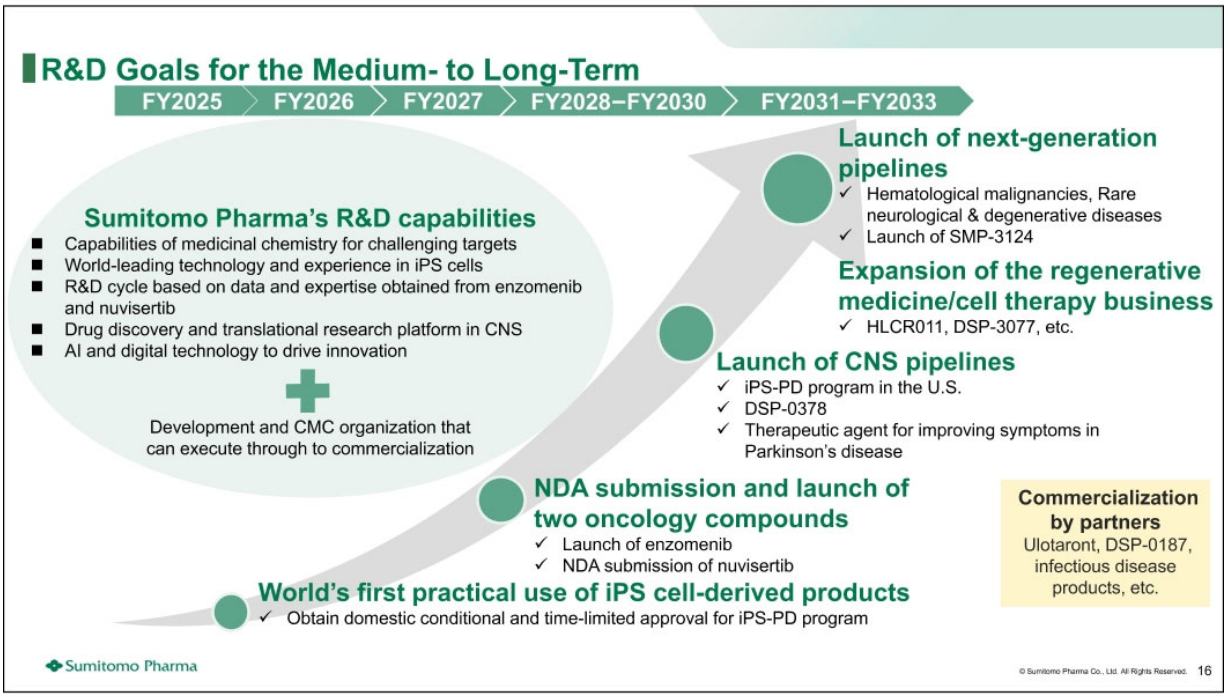


Here is the history of the discovery of DSP-0378.

Our experience with the older antiepileptic drug EXCEGRAN, as well as other development experience, has provided us with a library of compounds suitable for this classification, from which we found the starting compound for this agent.

We have also identified optimal compounds and points of action for refractory epilepsy through clinically relevant phenotypic screening and multiple pharmacological evaluation systems.

It acts on targeted GABAA receptors with clinically proven efficacy. We confirmed that this has a unique action that is different from existing drugs and also identified translational biomarkers. We are currently in the process of initiating a study to look at efficacy signals while confirming safety for patients.



Based on the above, the world's first practical application of iPS cell-derived products will be approved at the end of this fiscal year, with a time limit on the condition that it be in Japan. We hope to steadily proceed with Phase IV testing based on this.

In addition, we will steadily advance the development of two oncology products, enzomenib and nuvisertib, on which we are focusing our efforts, in order to bring them to the market as valuable drugs.

In the first half of the 2030s, we will continue development so that we can launch the iPS cell-PD program in the US, as well as DSP-0378, which I explained earlier, and a drug that improves the symptoms of Parkinson's disease.

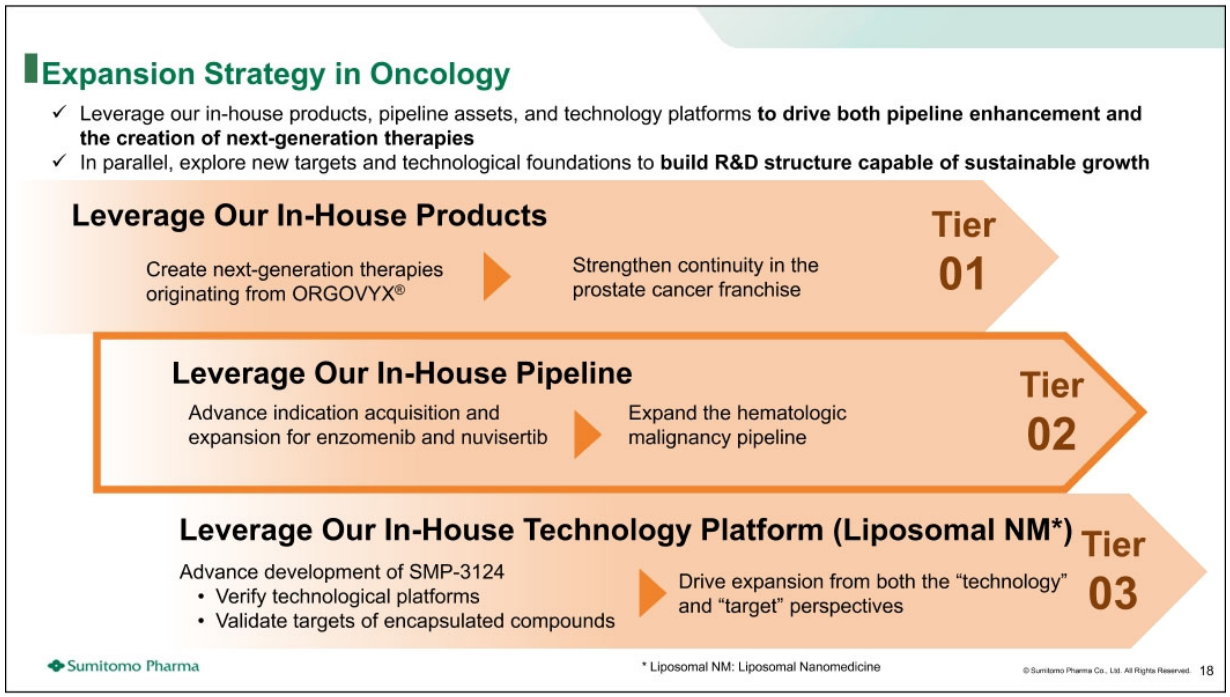
We will continue to make steady progress in the next generation of oncology and CNS products, as well as in the expansion of our regenerative and cellular medicine business, and make a strong contribution to the rebuilding of the value creation cycle of our group.

That's all from me. Thank you very much.

Kino: Thank you very much, Ms. Sato.

Next, Murata will explain about the two major oncology developments. Mr. Murata, please proceed.

Murata: My name is Murata, Global Oncology Strategy Lead. Thank you very much.

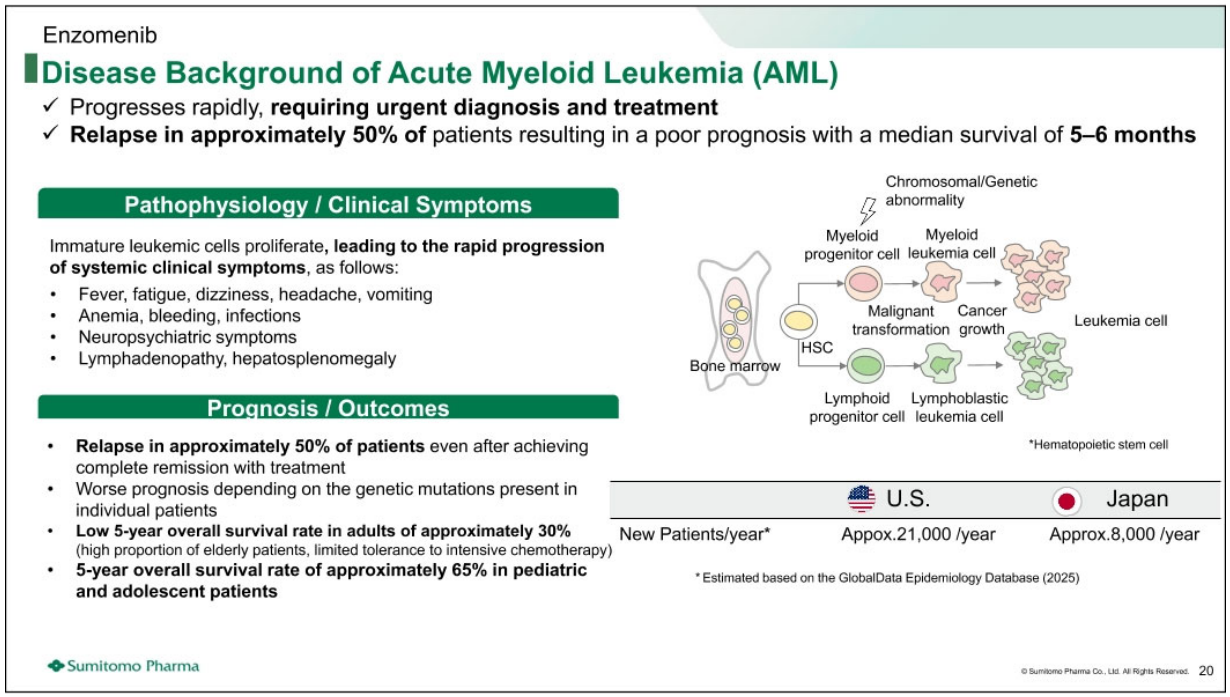


Sato has just introduced the expansion strategy for the cancer strategy. Today, I would like to introduce our efforts to obtain and expand the indications for enzomenib and nuvisertib in tier two, "Leverage our in-house pipeline."

As for data for enzomenib and nuvisertib, we are mainly using data presented at the American Society of Hematology meeting last December. So perhaps some of you have already heard about its contents. I would like to take this opportunity to explain again how we perceive the data and to present the possibilities of these two drugs.

The first is enzomenib.

The mechanism of action is selective menin inhibition. The development phase is currently in Phase II. The planned indication is acute leukemia. Among them, we believe that patients with acute leukemia, such as those with specific genetic mutations called KMT2A rearrangements and NPM1 mutations, will be targeted.

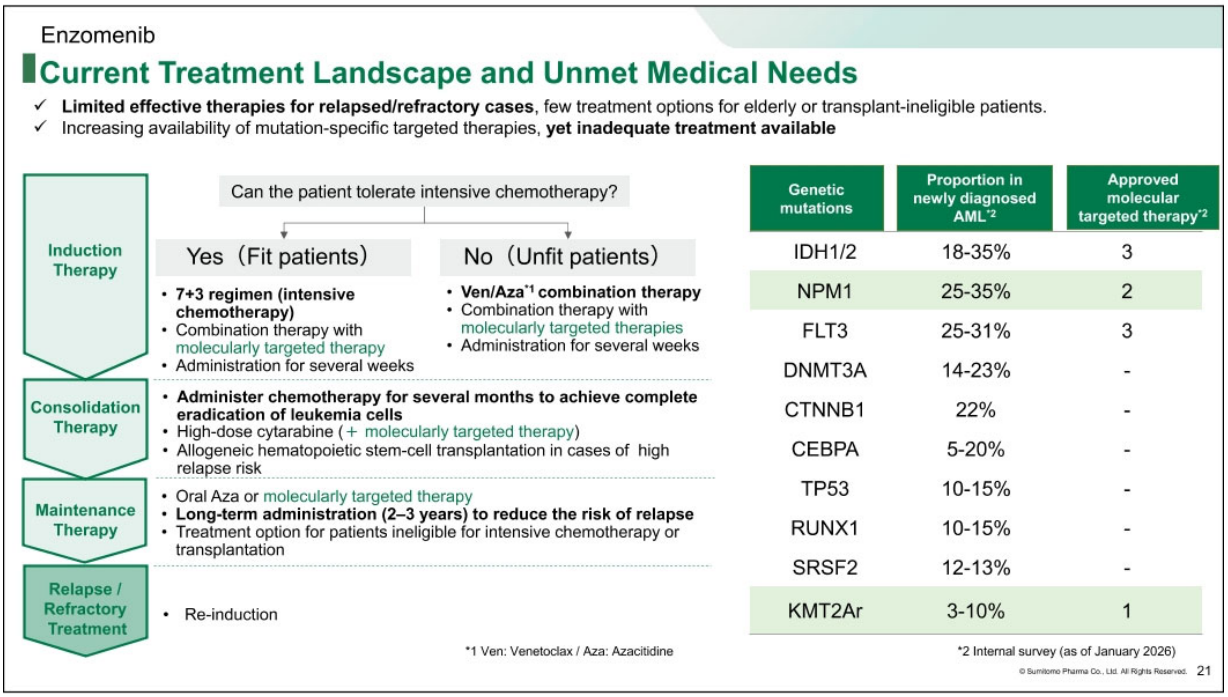


First, let me briefly introduce the diseases those cover.

We have summarized it here under the title Disease Background of Acute Myeloid Leukemia. The cause of this disease is that hematopoietic stem cells in the bone marrow contain progenitor cells that differentiate into myeloid cells, which become cancerous when accompanied by some genetic mutation, leading to leukemia.

As the name "acute" implies, this is an intractable disease that progresses very quickly and has a very poor prognosis if it relapses.

Although the number of new patients is small (21,000 in the US and 8,000 in Japan), once the disease develops, about 50% of patients will have recurrence, and the 5-year survival rate is about 30%, making it a disease with a very poor prognosis. So the medical need for this is very high.



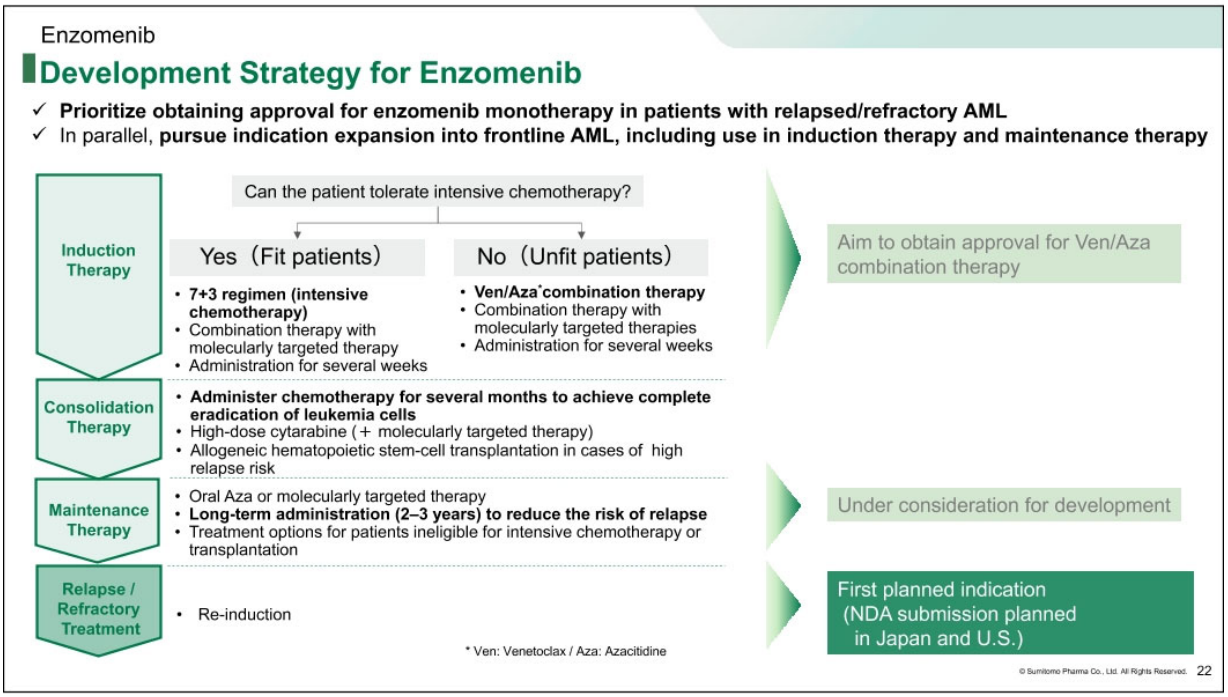
This is a summary of what treatments are currently being offered and how they are producing clinical results.

Basically, the treatment for acute leukemia, developed about 40 years ago, is to thoroughly tap the leukemia cells with a powerful anticancer drug. The treatment strategy is to achieve remission through this process, followed by bone marrow transplantation to restore normal hematopoietic function.

On the other hand, because powerful anticancer drugs are sometimes used, it is estimated that 5% to 10% of patients die as a result of treatment, and there is a high unmet need for patients who cannot tolerate strong anticancer drugs. Safer treatments may also be desired by patients who can tolerate strong anticancer drugs.

Listed to the right are the genetic mutations that cause acute leukemia. Since there is some overlap, the total is more than 100%. Several agents that act on some genetic mutations have been developed since around the 2010s and have been in clinical use since around 2015. IDH1/2 and FLT3 have been approved and used since the mid-2010s, but response rates for these treatments are also only 20% to 30%.

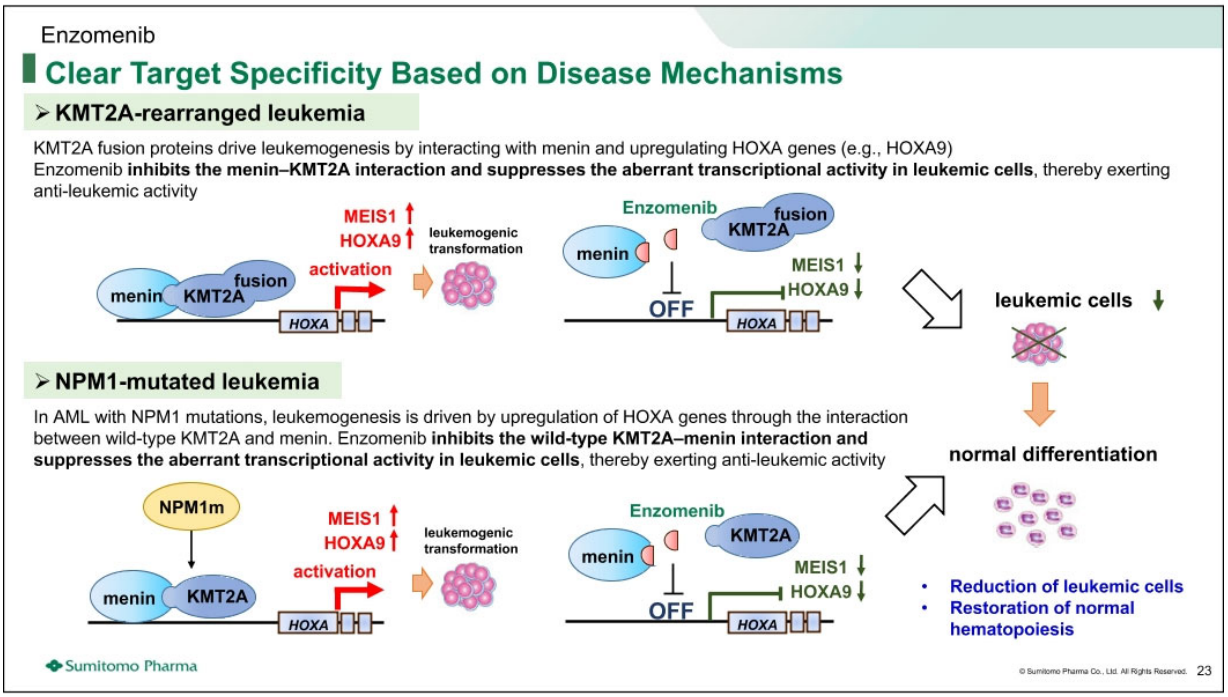
Although approved drugs for patients with NPM1 mutations and KMT2A reconstructions (the bottom line) have emerged in recent years, our analysis indicates that the level of treatment satisfaction is not high. So we believe that the medical need for this continues to exist.



Our initial strategy for enzomenib is to target the first indication based on the results of single-agent efficacy and safety studies in patients with relapsed/refractory disease.

After that, we would like to have enzomenib used to treat the entire leukemia. We would like to work on obtaining a three-drug combination indication for induction of remission therapy in patients with first-episode disease, particularly combining enzomenib with the two-drug combination of Venetoclax/Azacitidine.

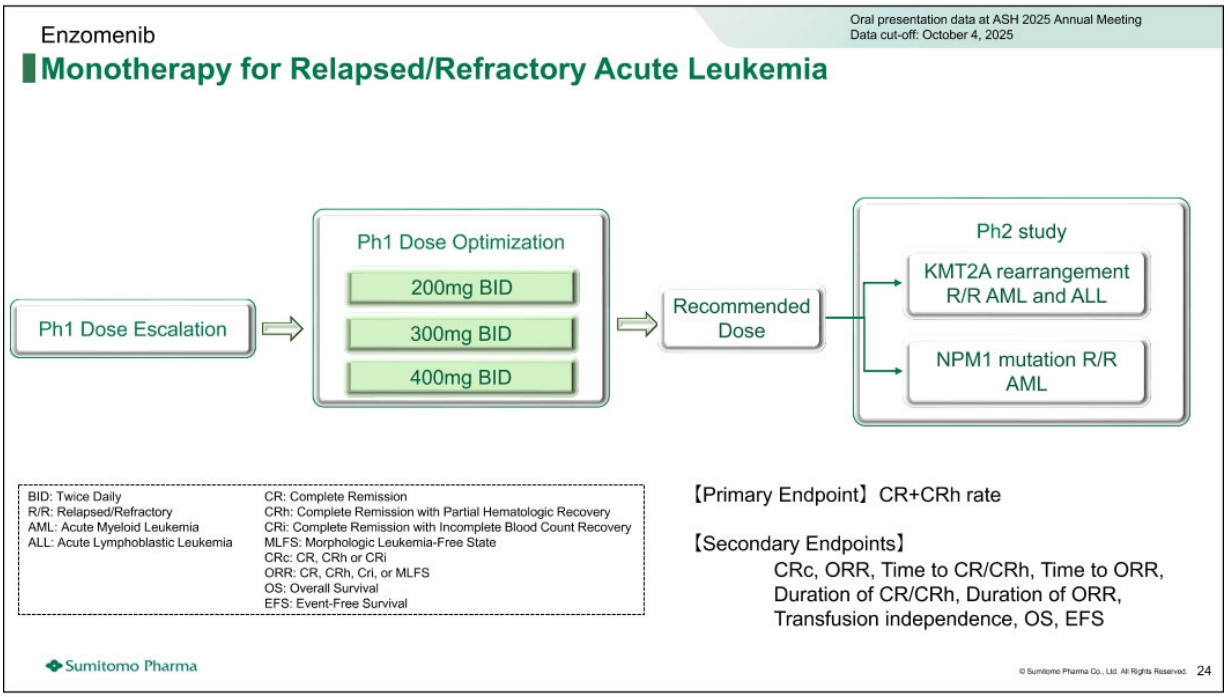
We also hope that enzomenib can be used for maintenance therapy leading up to bone marrow transplantation or continuing treatment to prolong the prognosis by maintaining the disease in remission for a longer period of time.



This page shows the mechanism of action of enzomenib.

Among the genetic mutations that cause leukemia, KMT2A-rearranged leukemia of menin is a mutation that fuses the molecule KMT2A with menin. In patients with this mutation, the structural activation of the gene cluster listed here causes the progenitor cells that would otherwise differentiate to stop differentiating and start proliferating, resulting in leukemogenesis.

Similarly in the NPM1 mutant form, NPM1 mutations in this area activate this function and lead to leukemia. The mechanism of action of enzomenib is to disengage this interaction between menin and KMT2A, thereby stopping the aberrant transcription program and normalizing the leukemia.

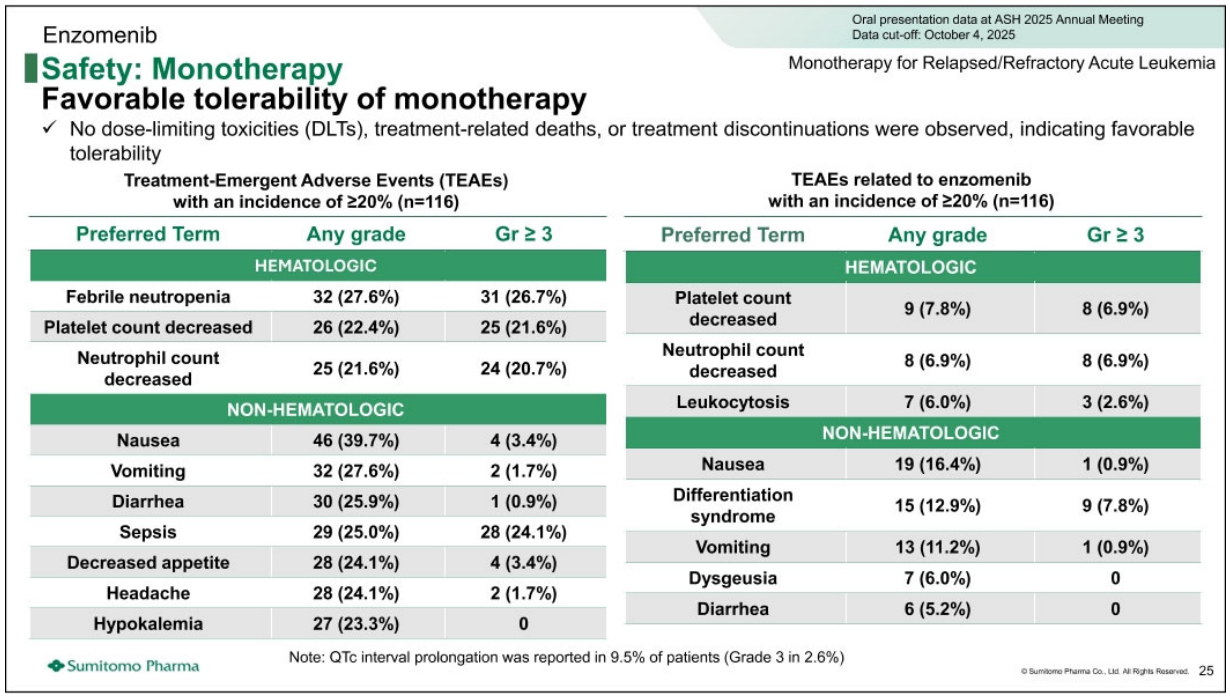


From here, I would like to introduce the clinical results.

The first is the results of a trial of monotherapy for relapsed/refractory leukemia.

The first part of the Phase I study is dose escalation, which is intended to evaluate what dose can be safely administered to a particular patient. After that, we will conduct a comparative study to determine the recommended dosage based on the Project Optimus guideline issued by the FDA. The recommended dose is determined by comparing three doses of 200 milligrams, 300 milligrams, and 400 milligrams per twice daily dose. The efficacy will then be evaluated in a Phase II study.

As I said at the beginning, the Phase II study is for patients with KMT2A rearrangements and patients with NPM1 mutations.

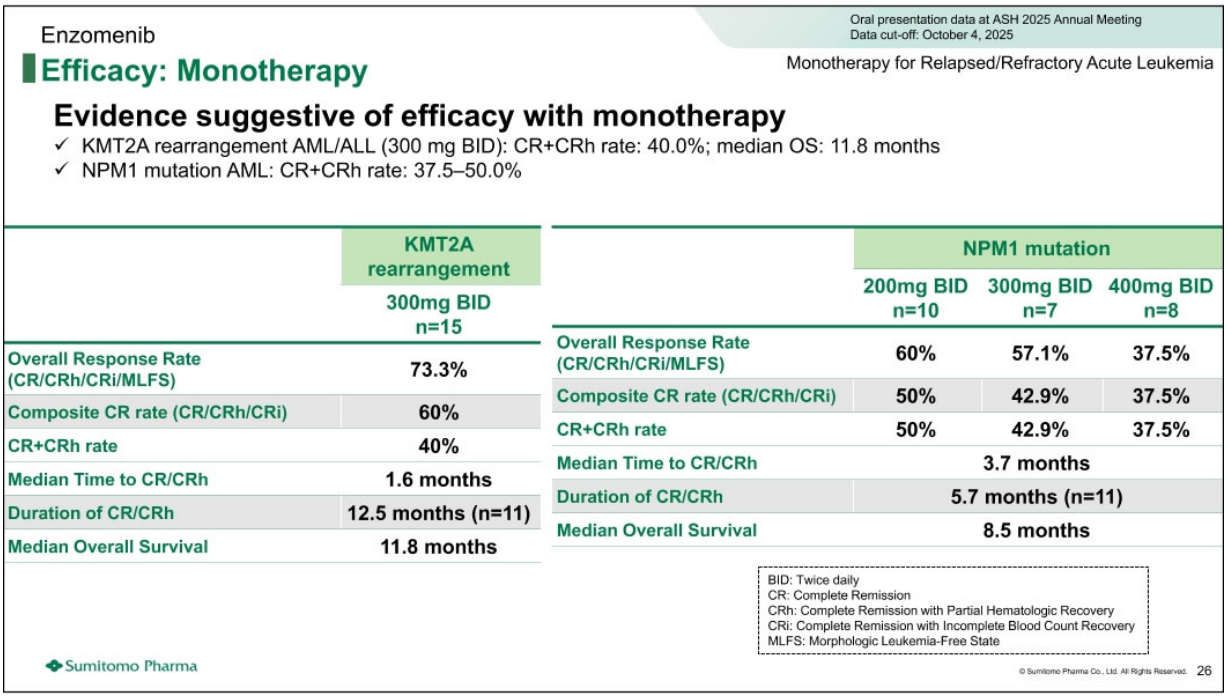


First, the results of the evaluation for safety, the objective of Phase I, are shown here.

The left-hand side summarizes adverse events with an incidence rate of 20% or greater, and the safety evaluation population includes all patients who participated in the study. There were 116 eligible patients as of last year's October 4 cutoff.

On the right are the adverse events that were observed and determined to be related to enzomenib. Only those with an incidence of 5% or more were picked up. The overall frequency of adverse events themselves is low, and no toxicities that could lead to dose-limiting toxicity have been observed in this dose-escalation study. Fortunately, there have been no cases of treatment-related deaths or discontinuation of treatment due to toxicity. Therefore, we believe that enzomenib can be safely administered and is well tolerated.

As Sato mentioned a little earlier in her explanation, we have also devised a way to avoid cardiotoxicity in designing this agent, and we have confirmed that the frequency of cardiotoxicity appearing is also very low.



Next are the results of single-agent efficacy.

On the left are the results of the KMT2A rearrangement test. The results of the study to determine the recommended dose as of the October 4 cutoff date were available, and 300 milligrams twice daily has been determined as the recommended dose. So the results we are showing you here are the results of a test at that capacity.

The response that will be evaluated as clinically significant for this trial is the Overall Response Rate at the top of the list. In short, in addition to the indicator of whether or not cancer cells are being reduced, what is really meaningful to the patient is what is called the Composite CR rate. This is an indicator that assesses whether leukemia cells are completely gone, together with whether normal hematopoiesis has been restored afterwards.

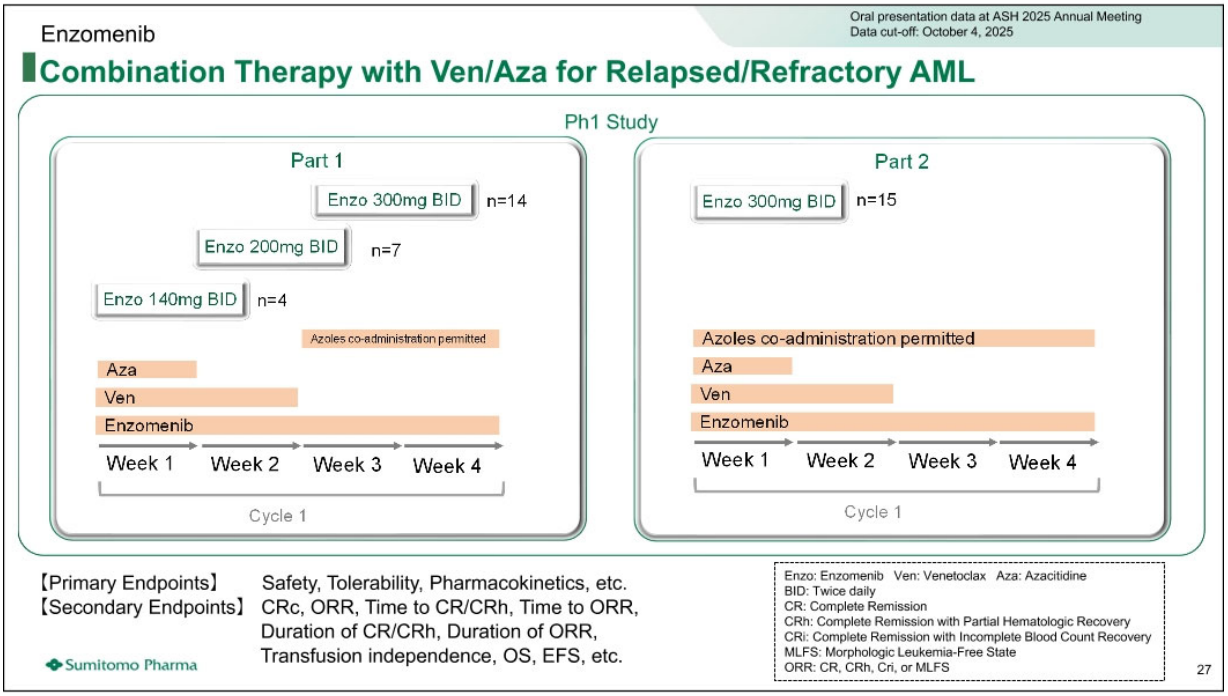
My apologies, CR + CRh rate below that.

The result is 40%. We consider the 40% result among patients with a very poor prognosis of KMT2A rearrangement, especially in patients with relapsed/refractory disease, to be a very encouraging result for us.

Another thing I would like to tell you is the Duration of CR/CRh. This is a measure of how long the status can be maintained after leukemia cells are gone. Although the number of cases is still small, we have confirmed that we have been able to maintain that status for about a year.

On the other hand, for the NPM1 mutation, we are still in the process of comparing three doses as of the October 4 cutoff. Among them, CR + CRh rate is about 50% to 37%, and Duration of CR/CRh is 5.7 months, although the evaluation period is short. We think this one is also promising to some extent.

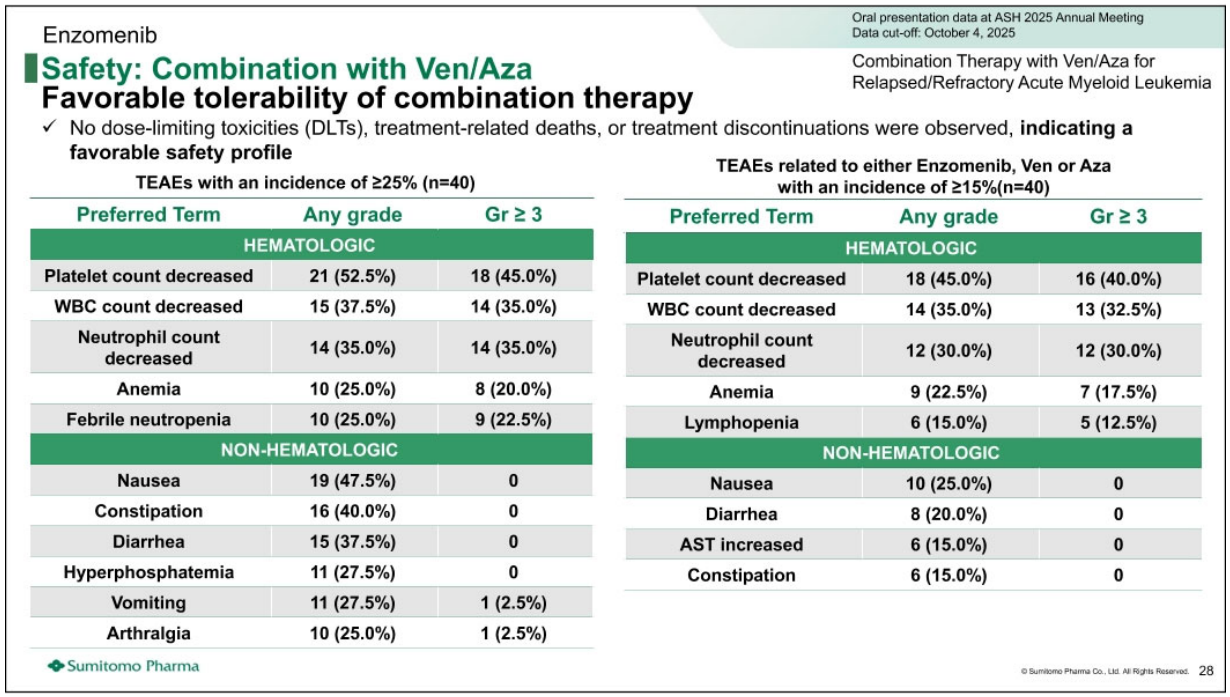
Based on these results, we believe that a single agent will provide a reasonable response. Currently, the efficacy is being tested in a Phase II study.



There is one more enzomenib data I would like to present. These are the results of a Phase I study of a three-drug combination of Venetoclax and Azacitidine plus enzomenib in patients with relapsed/refractory leukemia as a prelude to testing future indications in patients with first-episode AML.

This study is divided into part one and part two. In part one, the dose of enzomenib will be increased from 140 milligrams to 300 milligrams to see how well it is tolerated. In part two, we will [hoard the base] at 300 milligrams and check the response carefully.

In terms of the drug interactions of Venetoclax, part one is to check the safety of the three drugs while making sure that the duration of concomitant use of the azole antifungal agents does not overlap, and part two is to evaluate the efficacy and safety of the three drugs in combination while the azole is used in combination. We are doing this stepwise while taking safety into consideration.



Enzomenib

Oral presentation data at ASH 2025 Annual Meeting
Data cut-off: October 4, 2025

Safety: Combination with Ven/Aza
Favorable tolerability of combination therapy

Combination Therapy with Ven/Aza for Relapsed/Refractory Acute Myeloid Leukemia

✓ No dose-limiting toxicities (DLTs), treatment-related deaths, or treatment discontinuations were observed, **indicating a favorable safety profile**

TEAEs with an incidence of ≥25% (n=40)

Preferred Term	Any grade	Gr ≥ 3
HEMATOLOGIC		
Platelet count decreased	21 (52.5%)	18 (45.0%)
WBC count decreased	15 (37.5%)	14 (35.0%)
Neutrophil count decreased	14 (35.0%)	14 (35.0%)
Anemia	10 (25.0%)	8 (20.0%)
Febrile neutropenia	10 (25.0%)	9 (22.5%)
NON-HEMATOLOGIC		
Nausea	19 (47.5%)	0
Constipation	16 (40.0%)	0
Diarrhea	15 (37.5%)	0
Hyperphosphatemia	11 (27.5%)	0
Vomiting	11 (27.5%)	1 (2.5%)
Arthralgia	10 (25.0%)	1 (2.5%)

TEAEs related to either Enzomenib, Ven or Aza with an incidence of ≥15%(n=40)

Preferred Term	Any grade	Gr ≥ 3
HEMATOLOGIC		
Platelet count decreased	18 (45.0%)	16 (40.0%)
WBC count decreased	14 (35.0%)	13 (32.5%)
Neutrophil count decreased	12 (30.0%)	12 (30.0%)
Anemia	9 (22.5%)	7 (17.5%)
Lymphopenia	6 (15.0%)	5 (12.5%)
NON-HEMATOLOGIC		
Nausea	10 (25.0%)	0
Diarrhea	8 (20.0%)	0
AST increased	6 (15.0%)	0
Constipation	6 (15.0%)	0

Sumitomo Pharma

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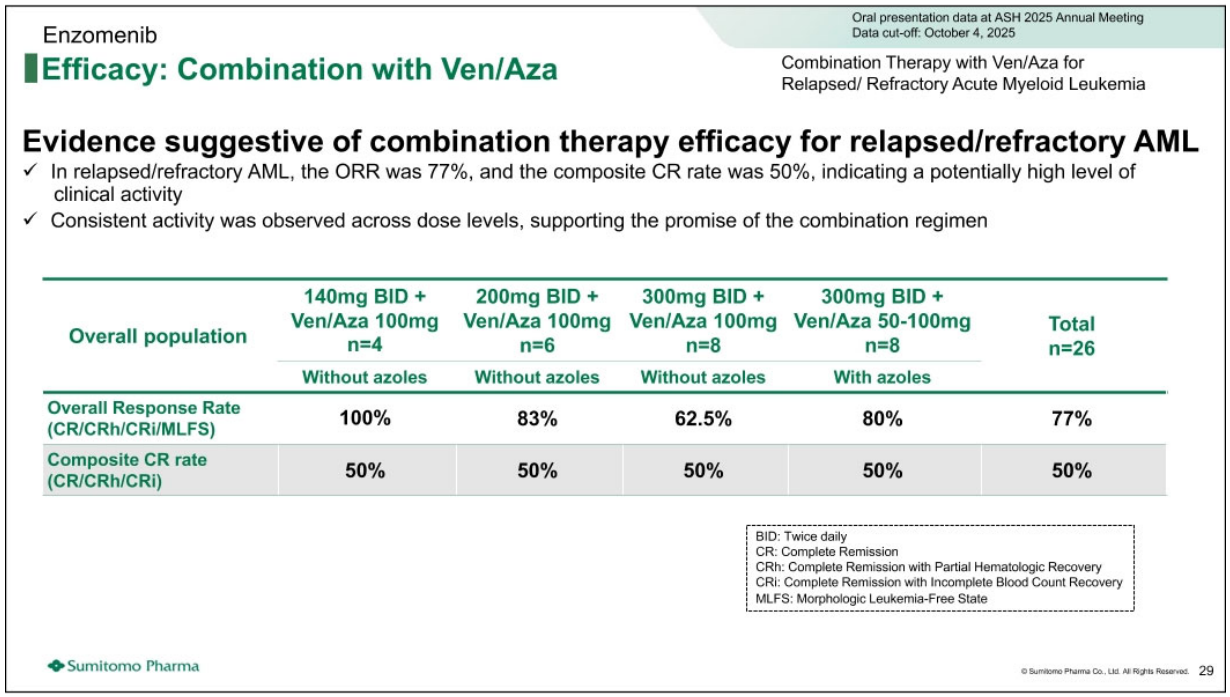
The next page also shows the safety results. The results of this evaluation are not for all patients, but for 40 patients who were administered in combination.

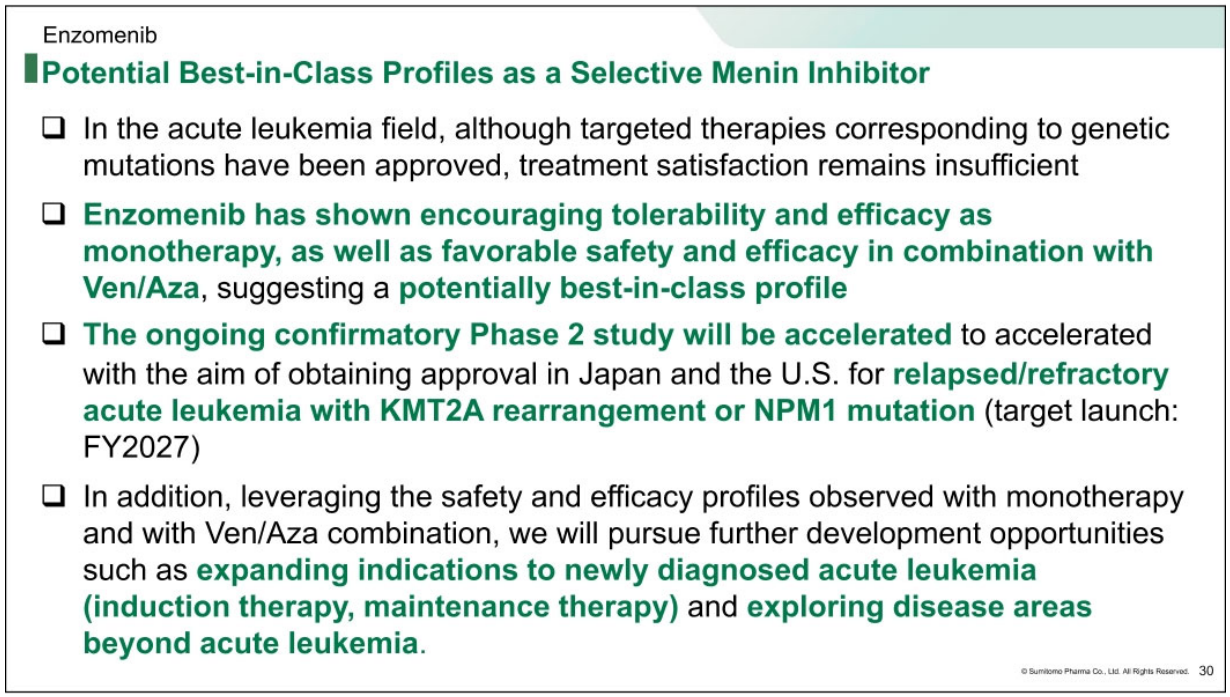
In both cases, adverse events related to either enzomenib or Venetoclax/Azacitidine occurred to the extent shown. There have been no additional adverse events with enzomenib beyond the adverse events seen with Venetoclax/Azacitidine, nor have there been any treatment-related deaths or events that would lead to discontinuation of the drug. It has been confirmed that the three drugs can be administered in combination.



Next is its effectiveness.

See CR rate as before. About half of the patients at all doses show a response of elimination of leukemia. While some patients were relapsed/refractory and relapsed on Venetoclax/Azacitidine, 50% of patients showed response, which we believe confirms a very high response rate.

This is a great encouragement to us. Based on this trial, we are now preparing to proceed to front-line and first-episode patient trials.



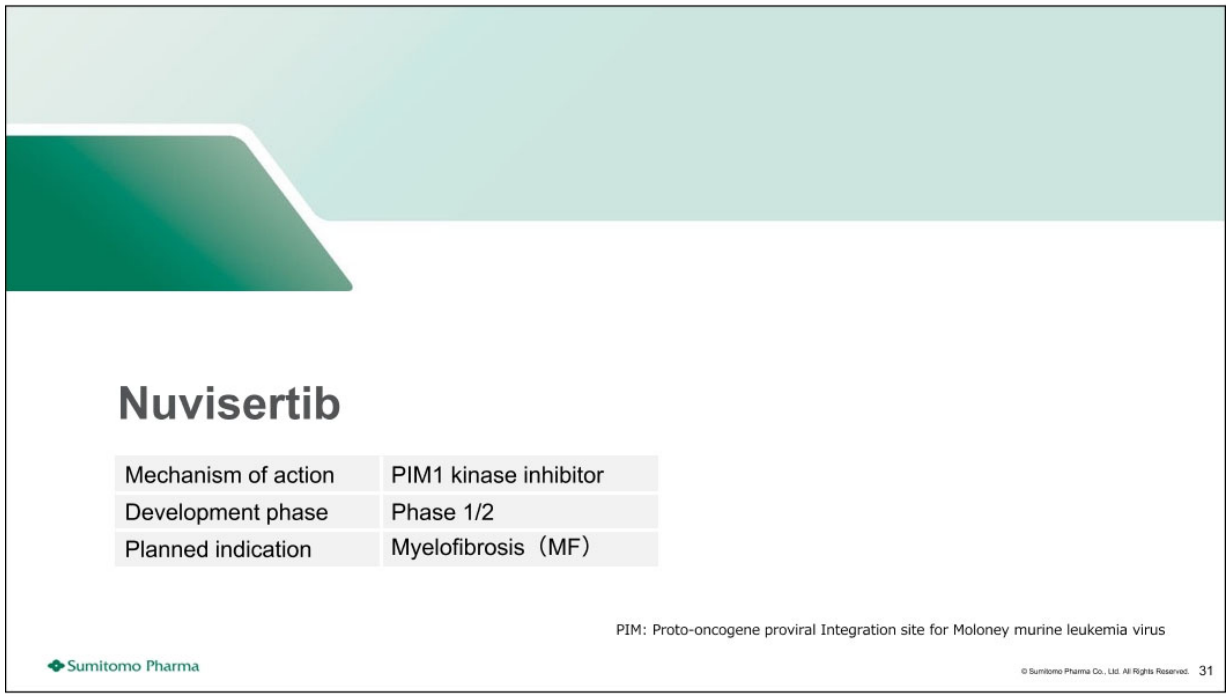
These are the descriptions of enzomenib. To summarize what I have explained so far, we recognize that in the area of acute leukemia, although therapeutic agents corresponding to genetic mutations have been sequentially approved, the level of treatment satisfaction remains insufficient.

In this context, we are beginning to confirm that enzomenib has efficacy and tolerability as a single agent in patients with certain genetic mutations, as well as efficacy and safety in combination with Venetoclax/Azacitidine. Therefore, we strongly believe that enzomenib has the potential to be a best-in-class menin inhibitor.

We will accelerate the validation Phase II study and aim to obtain approval in Japan and the US for relapsed/refractory acute leukemia.

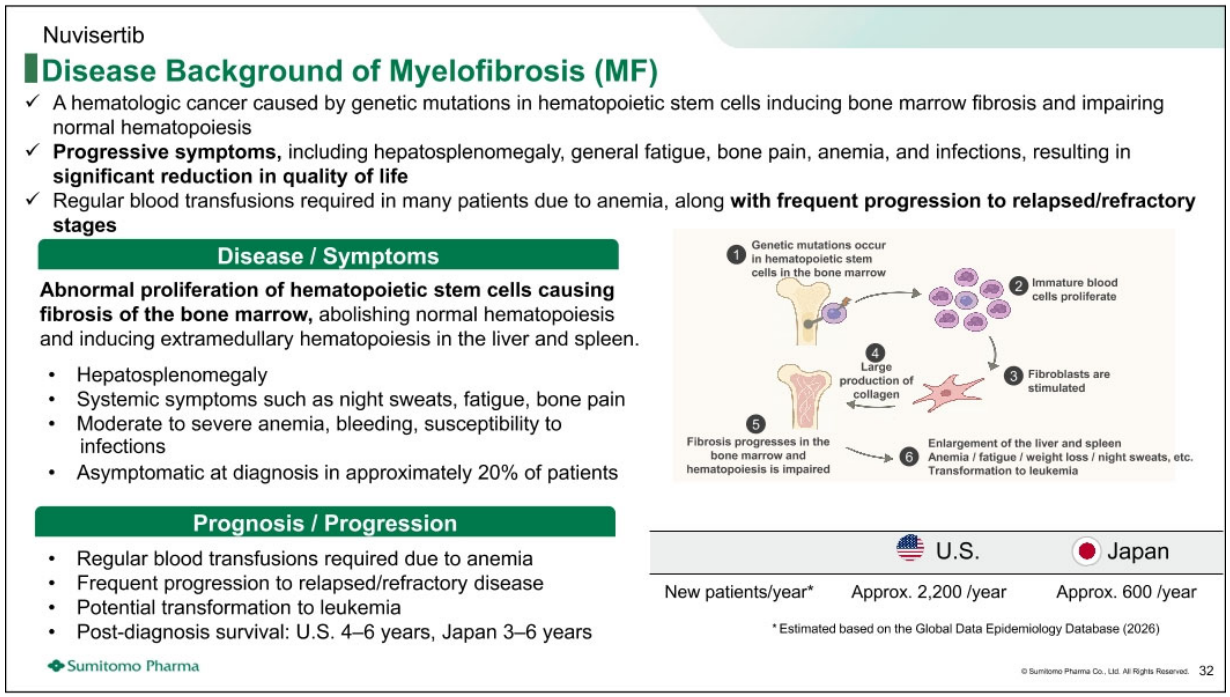
Furthermore, as I mentioned earlier, we would like to promote enzomenib by expanding its indication in the treatment of first-episode acute leukemia in combination with Venetoclax/Azacitidine, and by pursuing further development opportunities, such as expansion into diseases other than acute leukemia, where the menin molecule is associated with cancer.

These are the explanation of enzomenib.



I would like to move on to nuvisertib.

Its mechanism of action is inhibition of a kinase called PIM1. This is currently in Phase I/II study. The planned indication is a blood disorder called myelofibrosis.



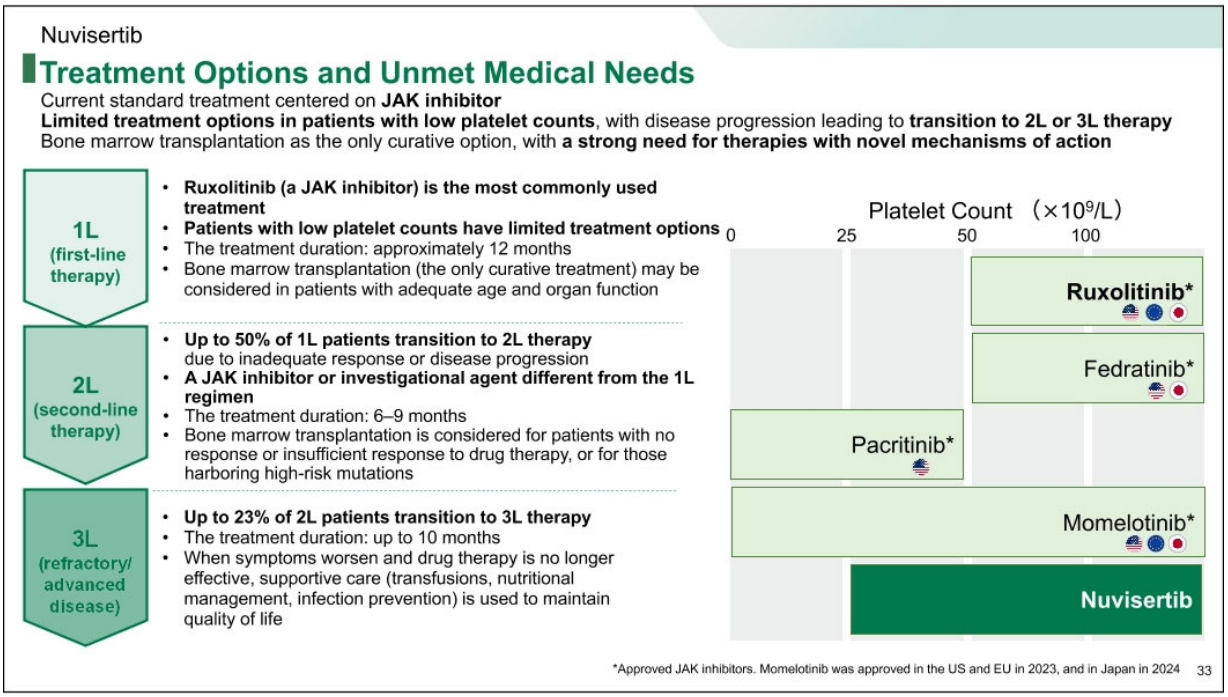
First, let me explain what myelofibrosis is.

This is another disease that occurs in the bone marrow. In the hematopoietic stem cells in the bone marrow, another genetic mutation occurs, primarily a mutation in JAK2, which results in an increase in immature blood cells, and the bone marrow itself becomes fibrotic with a large amount of collagen.

The result is extramedullary hematopoiesis in the liver and spleen outside of the bone marrow because normal hematopoiesis cannot occur in the bone marrow, a tissue very important for hematopoiesis. This can result in symptoms such as swelling of the liver and spleen, an increase in immature blood cells and a decrease in normal blood cells, anemia, inability to stop bleeding when bleeding, and susceptibility to infection.

The number of new cases of this disease is very small: 2,200 new cases per year in the United States and about 600 in Japan. However, symptoms can be difficult to recognize, and about 20% of patients have no symptoms at the time of diagnosis. This is a disease that is difficult to detect, such that some may find out they have the disease when they are diagnosed with anemia during a physical examination.

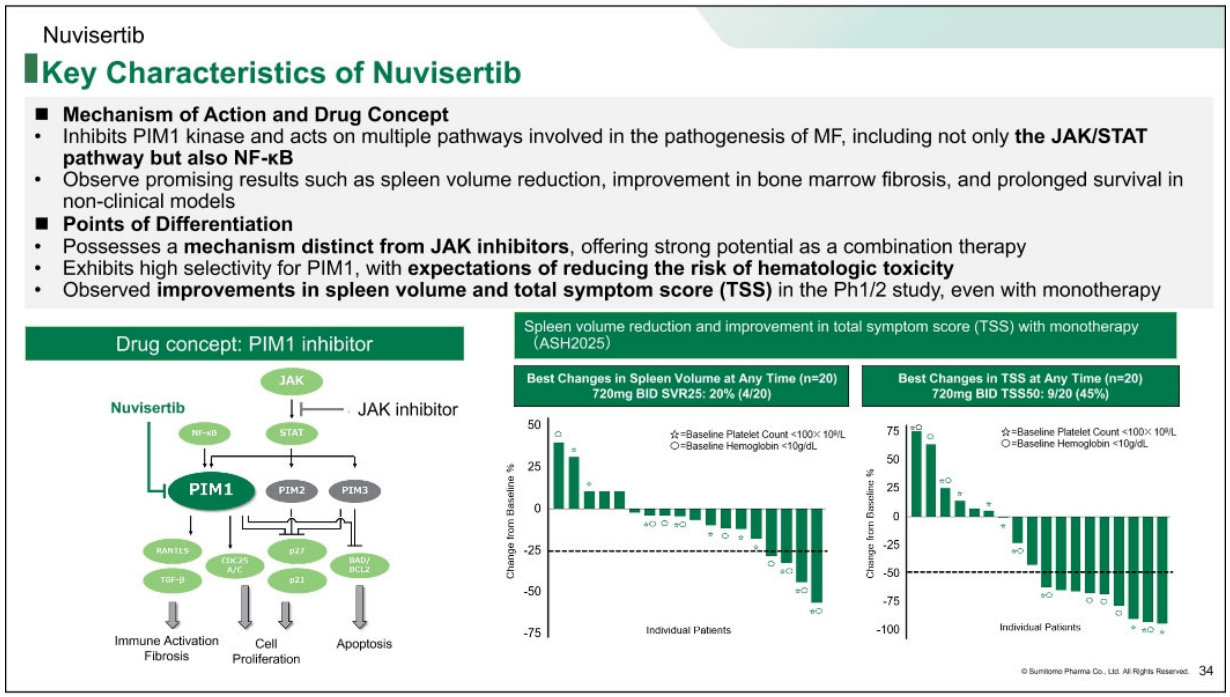
Treatment involves improving anemia through blood transfusions and other means, as we will briefly discuss later. However, some patients gradually become refractory and eventually develop leukemia. Based on this epidemiological data, the prognosis in the United States is 4 to six years after the diagnosis is confirmed, while in Japan it is three to six years.



The current main treatment for myelofibrosis is ruxolitinib, an inhibitor of JAK, the gene responsible for the disease, which was approved around 2010.

For a long time, no drug other than ruxolitinib had been approved, and despite its hematologic toxicity and other side effects, there was no other option but to use ruxolitinib. Recently, however, another JAK inhibitor has been approved, and there are now a few more treatment options.

However, there is no therapeutic drug with a mechanism of action other than JAK, and JAK inhibitors are difficult to use in anemic patients, so we believe there is an unmet need in this area. We believe that our nuvisertib has value as a complementary treatment.



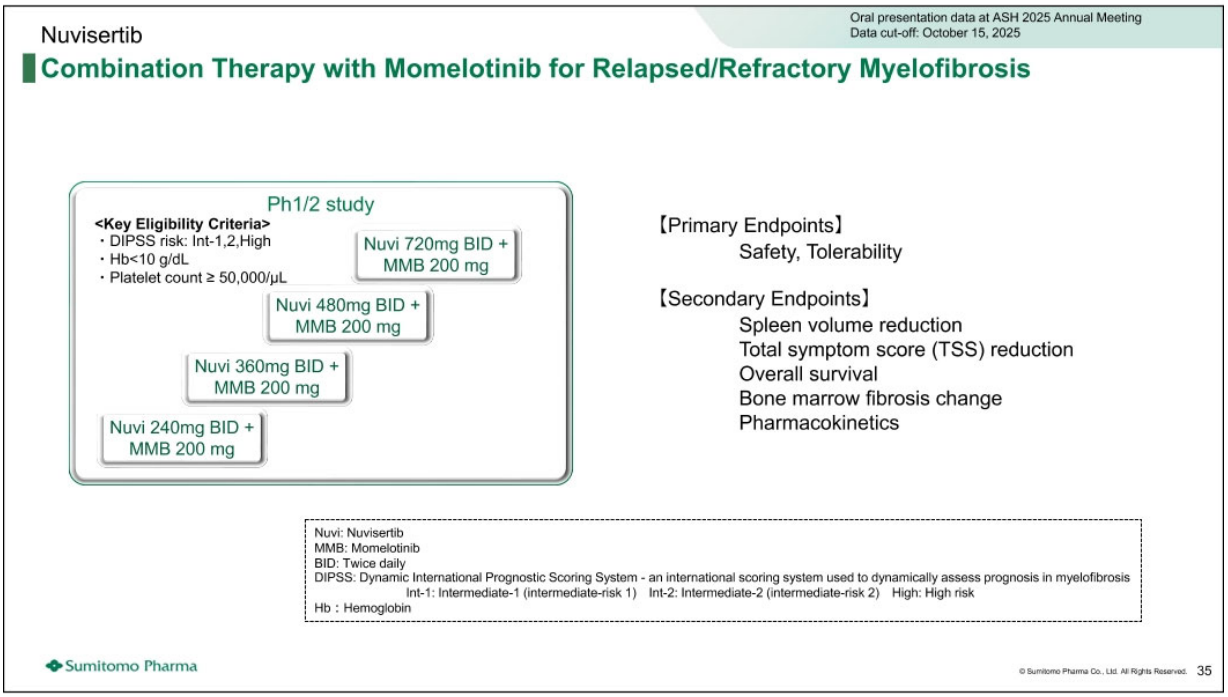
Nuvisertib, as I mentioned at the beginning, is an inhibitor of PIM1. PIM1 is a protein located downstream of the JAK signaling pathway that is responsible for the development of myelofibrosis. The JAK signal is divided into several branches, one of which leads to fibrosis, and PIM1 is a factor that is particularly closely related to fibrosis.

This has been confirmed in nonclinical knockout mouse experiments and other studies. What is expected from PIM1 inhibitors is work to selectively stop fibrosis, rather than the broad inhibitory effects that emerge with JAK inhibitors.

In addition to JAK, other factors that affect PIM1 include inflammation-related signals such as NF-Kb. Mechanistically, it is also expected to have effects that cannot be obtained with JAK inhibitors.

On the right are the results of the clinical efficacy of a single agent, as presented at the American Society of Hematology meeting. On the left is the effect of the reduction of the spleen. One of the symptoms of patients with myelofibrosis is an enlarged spleen. This is an assessment of how small it would be with a single agent. We have confirmed that for patients above a certain level, the reduction of the spleen can be observed with a single agent.

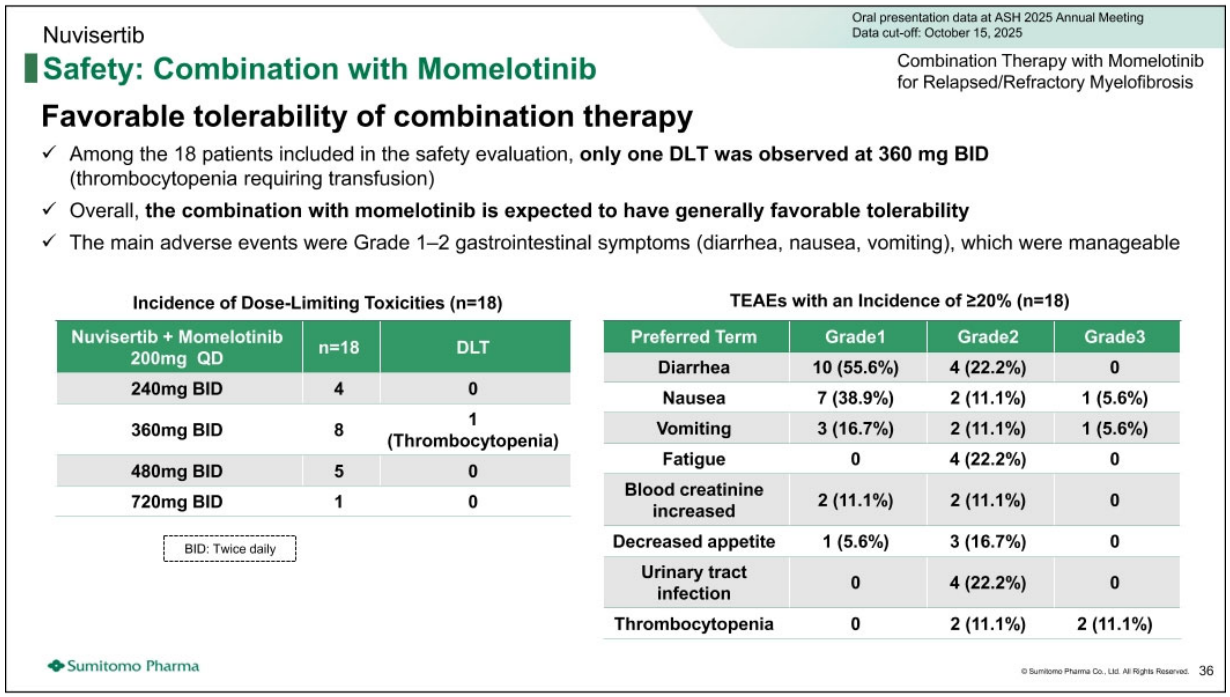
The right side shows the extent to which the various symptoms associated with myelofibrosis, which I mentioned earlier, have improved. Scores have improved by more than 50% in many patients, and the effectiveness in improving clinical symptoms has been recognized.



In this context, we also considered maximizing the features of nuvisertib. We are conducting a Phase I/II study in patients with relapsed/refractory myelofibrosis in combination with momelotinib, a JAK inhibitor that is relatively easy to use for platelet depletion, to see if the combination could take advantage of our agent's characteristics.

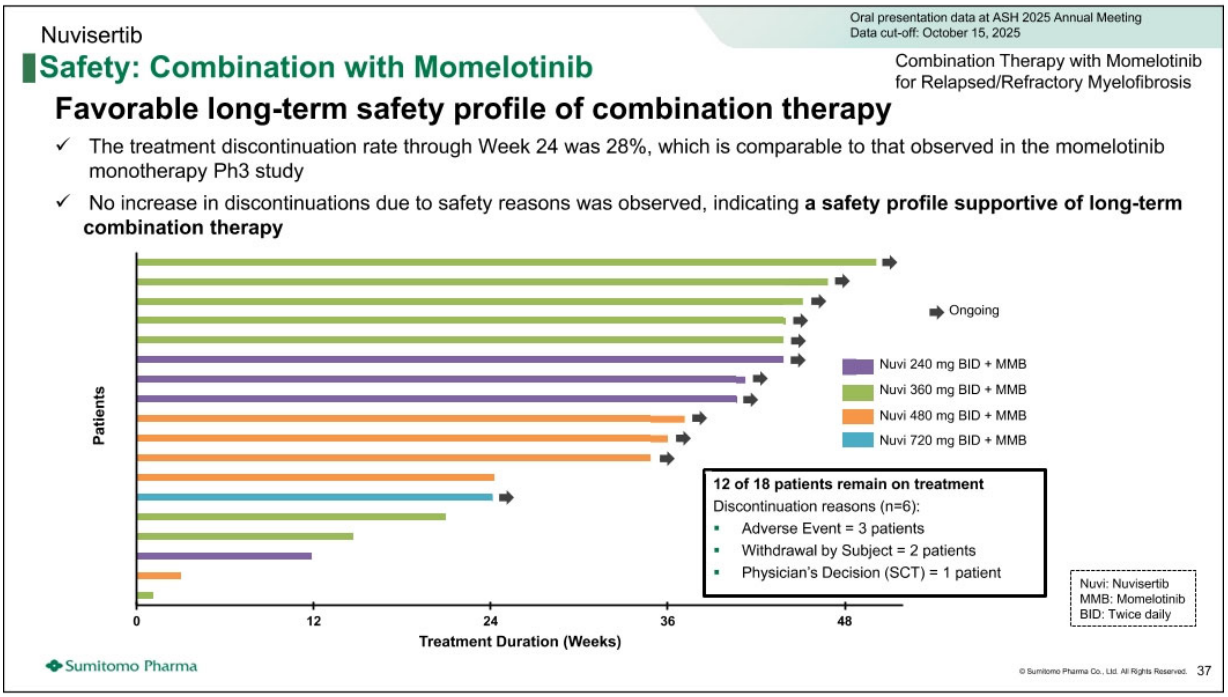
In Phase I, the approved momelotinib volume of 200 milligrams will be combined with nuvisertib in doses ranging from 240 to 720 milligrams twice daily to ensure safety and tolerability.

In addition, secondary endpoints will include the effect on the spleen, systemic symptom scores, and overall survival.



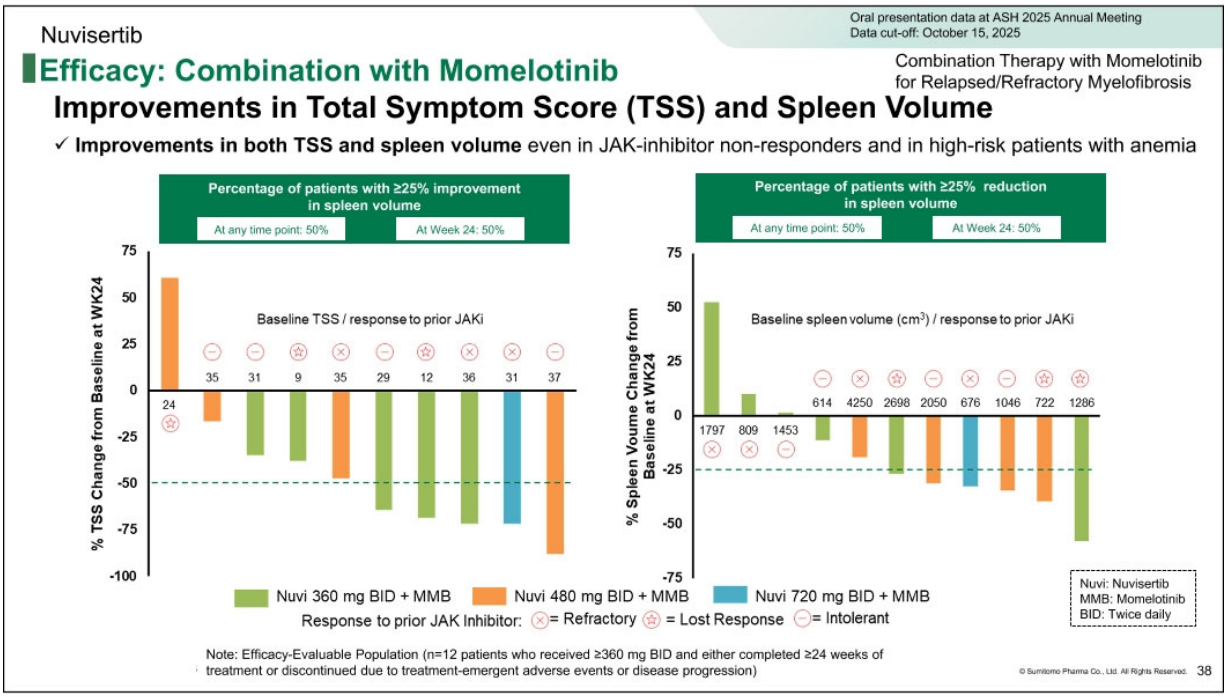
First, regarding safety, this is data from 18 patients that can be administered and evaluated. Although one patient is showing symptoms of DLT, we believe that it is basically well tolerated.

The adverse events observed were relatively mild, either grade one or grade two, and we do not foresee any problems with the combination with momelotinib.



These are the results of a test to confirm whether the product could be administered over a long period of time.

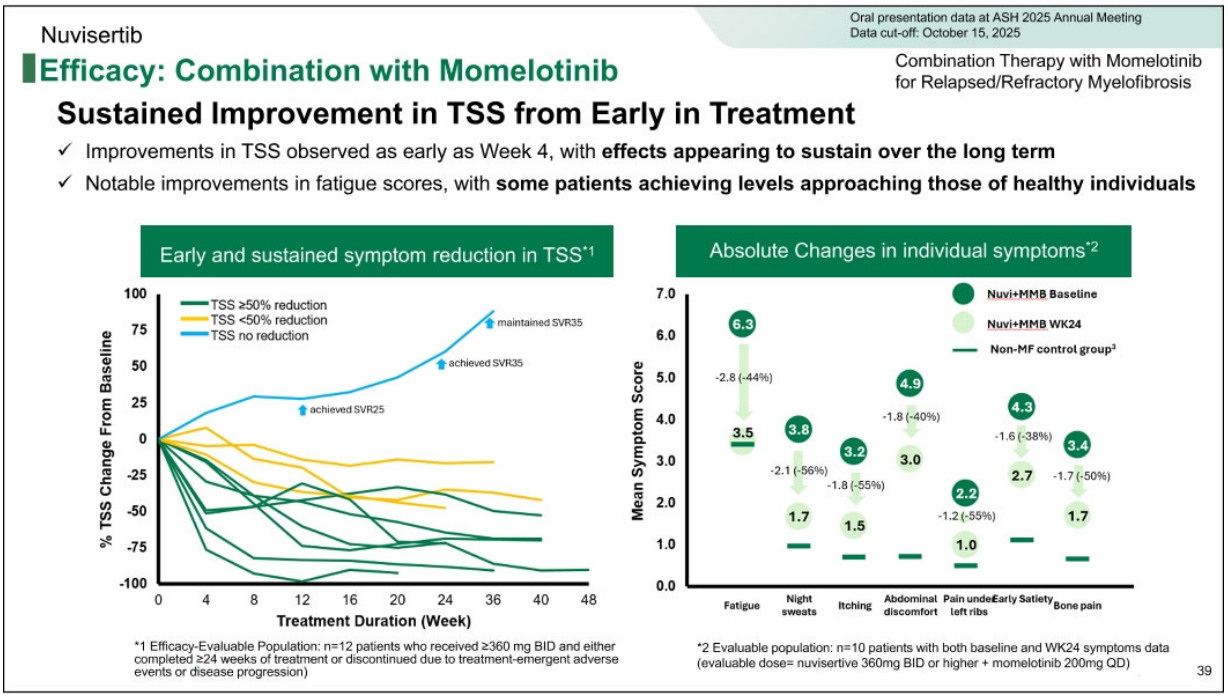
The horizontal axis is the administration period. The majority of patients are able to receive the drug up to around 24 weeks, with a discontinuation rate of 28%. A Phase III study, the MOMENTUM study of monotherapy with momelotinib alone, did not show a higher discontinuation rate than this study, which also confirmed that the safety of the combination is not compromised.



This is an evaluation of the effectiveness of the product under combined use.

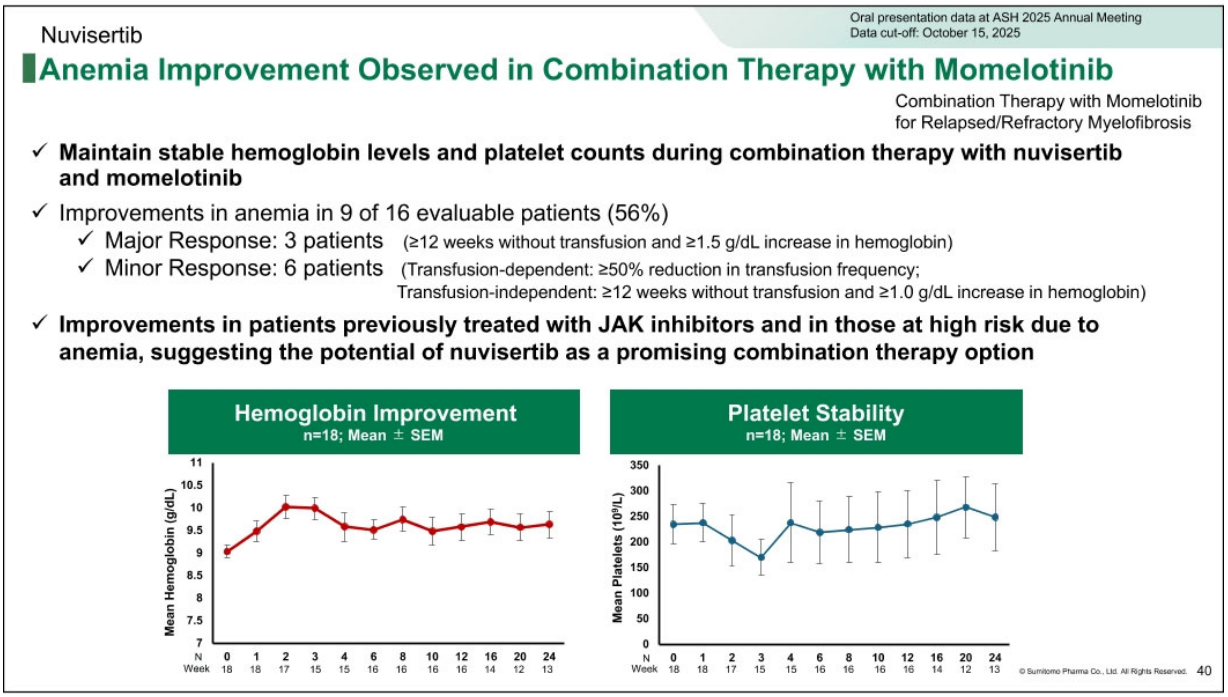
The left figure shows the improvement of total symptoms and the right figure shows the effect on the size of the spleen. Various red markings are written on it. Patients in this study are those who have relapsed. Patients who responded once to a JAK inhibitor and relapsed, or who did not respond in the first place, or who were unable to receive the drug due to side effects, are being invited to participate in this trial.

We believe that the combination of the two drugs has shown excellent efficacy in improving total symptoms and reducing the size of the spleen.



Here is a more detailed view of the scores over time and what kind of scores are changing.

What we can tell you is that the major scores improved relatively quickly and that it has been sustained for a long time. Improvements in total symptom scores are not merely small numerical improvements, but improvements in a wide range of items to the level of scores that a healthy person would show, as indicated by the horizontal bar. We recognize that by using the combination of the two, we can see the effect of improving not only one point, but the whole.

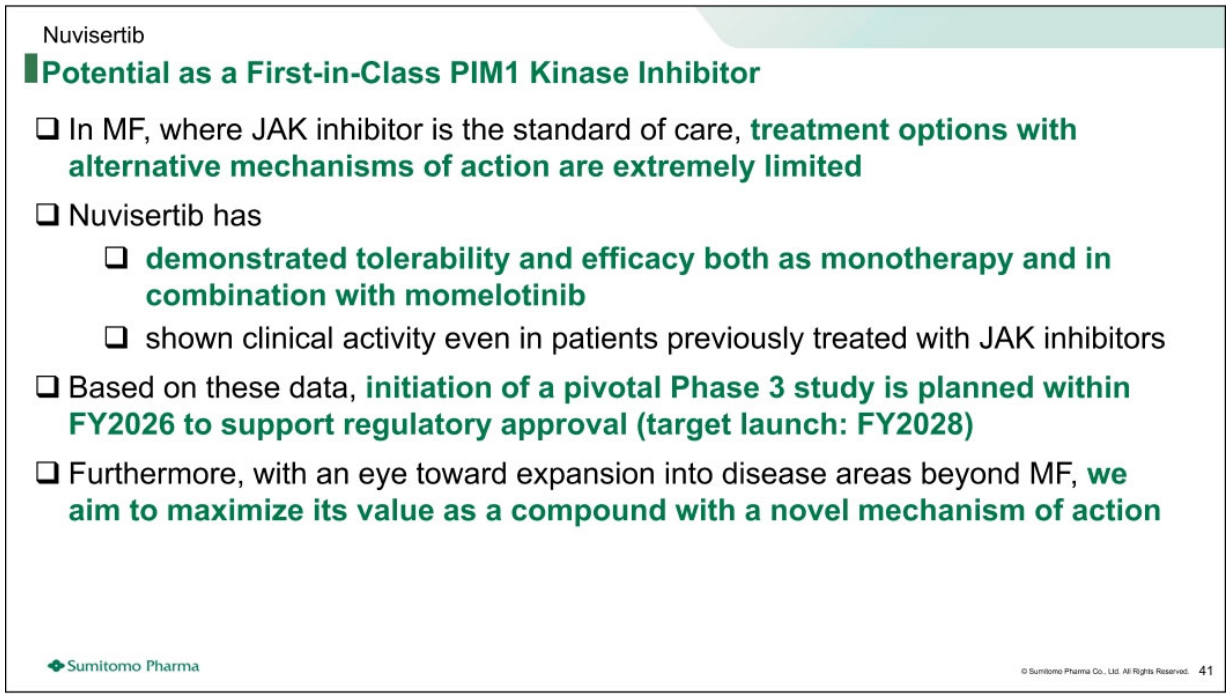


Here are data showing the effect of momelotinib combination on improving anemia.

We have been able to confirm that hemoglobin levels and platelet levels have remained stable without any decrease due to the combination.

In addition, three patients had dramatic improvement in their anemia symptoms, such that patients who had needed blood transfusions no longer needed them. Even with the 6 patients here, we were able to cut the frequency of blood transfusions into less than half.

The improvement in anemia is also very significant for the patient's quality of life. Improvement effects have been observed in these areas, and we believe nuvisertib is promising as an adjunctive therapy.

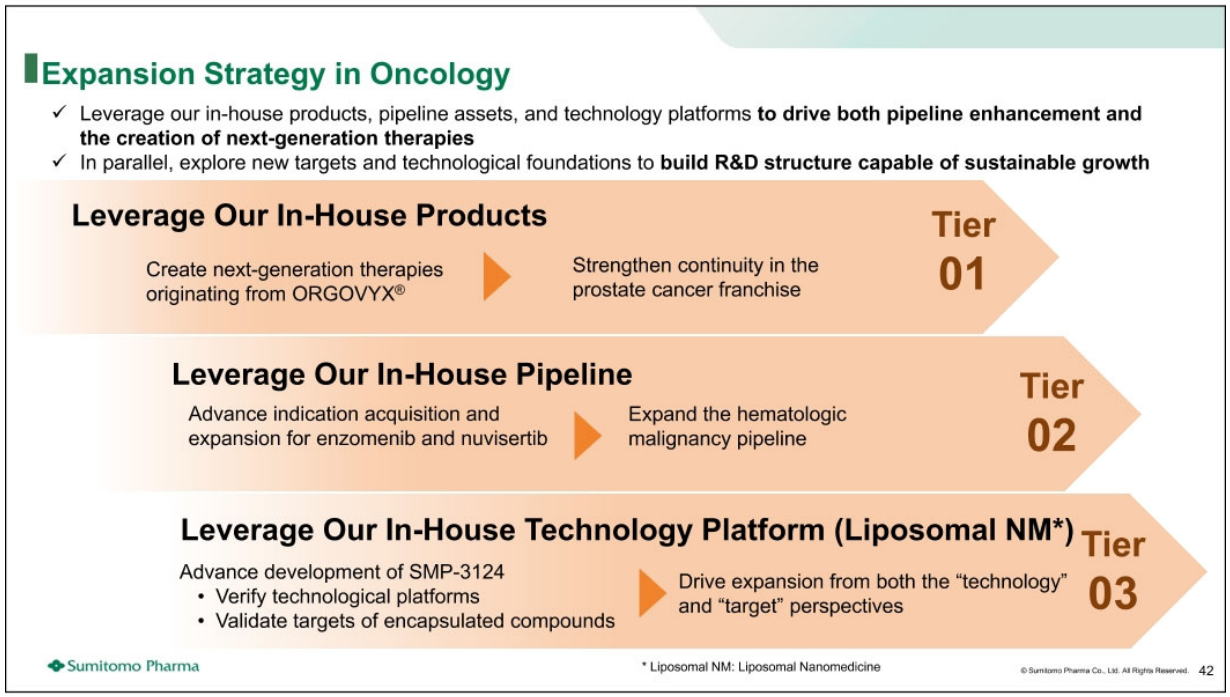


I will summarize the above. There is no drug approved in the world as a PIM1 inhibitor. If we can move forward with this trial and get it approved, we may be able to demonstrate the potential of this drug to become a first-in-class drug.

I believe that we will be able to make a very significant contribution to patients with myelofibrosis by providing them with a treatment option that has a different mechanism of action.

As for nuvisertib, as I mentioned earlier, we are in the process of confirming its tolerability and efficacy as a single agent or in combination with momelotinib. Based on these data, we plan to start a validation Phase III trial this fiscal year to provide the basis for an application for approval, with the aim of bringing the product to market as soon as possible.

Since we were talking about myelofibrosis as a disease, fibrosis was the key to the mechanism of action. We believe that we will be able to target diseases other than myelofibrosis. We intend to maximize the value of the novel mechanism of action in accordance with the biology of PIM1.



This is the last slide.

As our strategy for expansion in the oncology field, today I have introduced tier two, "Leverage our in-house pipeline." There are also tier one and tier three activities. We look forward to continuing to make solid progress in oncology R&D.

Thank you very much.

Kino: Thank you very much, Mr. Murata.

Question & Answer

Kino [M]: We will now answer questions from analysts and investors. The end time for Q&A will be 15:20.

Mr. Muraoka of Morgan Stanley MUFG Securities, please proceed.

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

First, let me ask about enzomenib. Sorry, I don't have a basic understanding, so let me ask a basic question. I kind of understand the concept of combination therapy, mainly in combination with Ven/Aza. Should you not aim to use it in combination with 7+3 regimen, or is it not a good match?

Murata [A]: we expect 7+3 regimen will continue to be used by patients as a high intensity chemotherapy. So I think it is necessary to continue to examine what kind of effect can be further expected when enzomenib is used in combination with 7+3 regimen.

Muraoka [Q]: In other words, you are also going to continue the study of enzomenib in combination with 7+3 regimen, right? This slide appears to be intended that you are focusing the combination therapy with Ven/Aza.

Murata [A]: I will add some background. The doctors are doing a lot of testing to see how much benefit the combination of Venetoclax/Azacitidine has for patients compared to the 7+3 regimen. Some of the results were also presented at last year's American Society of Hematology (AS) meeting.

As evidence accumulates, we expect that Venetoclax/Azacitidine may become the overall standard of care. However, this is not something that will suddenly change. The 7+3 regimen, of course, has its risks, but it has been well established for over 40 years, and I do not expect it to go away anytime soon.

In this context, we believe it is necessary to examine how enzomenib can be used, present data, and if it can be used, promote it as well.

Muraoka [Q]: Thank you.

One more thing about nuvisertib. This may be a bit of a business-oriented question, even though it is an R&D meeting. I think many people are probably wondering if GSK is the future partner when the combination with momelotinib is going so well. Considering that Jakavi is the standard of care and its patent expires in 2028 to 2029, you may not be able to fully exploit the potential of this drug if you only do the momelotinib combination. You may have to be aware of the ruxolitinib combination as well. But if you were to work with GSK, I imagine that would not be the case. Is this a skewed view?

Murata [A]: We are currently confirming the tolerability of the combination with ruxolitinib in a Phase I study. We would like to consider how to proceed with the verification after accumulating more data on how it can be used in combination with ruxolitinib.

Sato [A]: Regarding partners, GSK is not the only one.

Muraoka [Q]: Sorry, it was not a good idea to ask the question whether you would decide on GSK or not. Is it wrong to think that when ruxolitinib becomes generic in 2028 or 2029 or so, the combination with ruxolitinib must be verified to exploit nuvisertib's full potential?

Murata [A]: I think you make a very astute point. For many years, ruxolitinib has been used as the golden standard, and clinical doctors have a great deal of experience with the drug. It is a really commonly used drug, so I think it is very important to know if it can be used in combination with such drugs.

On the other hand, as shown in the slide, ruxolitinib has the problem that it cannot be used in patients with low platelet counts due to hematologic toxicity. Considering that this disease itself is a disease that causes anemia and low platelet counts, I am hopeful that momelotinib, which is widely available, may become the standard in the future.

Against this background, we were among the first to combine the drug with momelotinib. We do not deny the possibility of ruxolitinib, but for the first time we were able to show data on its use in combination with momelotinib, and we thought it was very valuable, so we presented it at the American Society of Hematology meeting.

Muraoka [Q]: Thank you.

One more thing about tier one of the oncology expansion strategy on page 10. I am not sure what you mean by "ensure continuity in the prostate cancer franchise" as an extension of ORGOVYX. I don't see many drugs in the area of prostate cancer in your pipeline list, what do you mean?

Sato [A]: The pipeline table certainly includes products with the clinical entries. What we mean here is that we are working with the intention of bringing a pipeline to the world that will succeed ORGOVYX while it is still in business period.

In our normal presentations, we do not explain much about compounds that have not yet entered clinical trials because there are still some uncertainties. This time, we have gone a step further and explained that we are engaged in such activities.

Muraoka [Q]: Thank you. In other words, we can expect to hear some interesting stories in the future in the area of prostate cancer, a solid cancer, at a briefing.

Sato [A]: Yes. We would like to proceed so that we can do so.

Muraoka [M]: I understand. Thank you very much. That's all from me.

Kino [M]: Thank you very much.

Mr. Wakao from JPMorgan Securities, please proceed.

Wakao [Q]: My name is Wakao from JPMorgan.

The first is about enzomenib. I would like to know your company's view on the concurrent data presented at ASH. I would like to know your assessment of whether this data, with comparisons to competing products in terms of safety, etc., makes them a competitive advantage. I think the monotherapy data put out so far has been very good compared to ziftomenib. On the other hand, regarding the relapse/refractory data for enzomenib and the Ven/Aza data, it is difficult to make an apples-to-apples comparison because the number of cases is not very large, but I think the efficacy is on the same level as ziftomenib. On the other hand, if I understand correctly, there has been QTc interval prolongation and I am concerned about safety issues. What is your company's current assessment of this data regarding this combination therapy?

Murata [A]: Thank you for your question.

It is difficult to make an apples-to-apples comparison of the combined data. Since the patients in our combination study were relapsed/refractory patients, it was originally difficult to see a response, and we had to combine the three drugs Venetoclax/Azacitidine and enzomenib. In this context, the CR rate is about 50%.

Right now, both NPM1 and KMT2A collectively are at 50%. Without accumulating a little more data on each patient, I think it is difficult to answer definitively how much difference there is. At this point, we do not believe it is significantly inferior to other companies. We are also hopeful that depending on future data, we may have a good enough chance of winning, or even a solid competitive edge.

Regarding the concern about cardiotoxicity, I think it is necessary to carefully evaluate the data, since they are recurrent patients. The target patients for the final combination with Venetoclax/Azacitidine are assumed to be patients with first-episode of the disease. We would like to examine how well it is tolerated in patients with first-episode disease in the future, and we would like to determine this very carefully.

Wakao [Q]: I believe the other company's product for ziftomenib did not cause QTc interval prolongation in the Ven/Aza data, in a similar patient population. For your company QTc interval prolongation was seen, is this considered an inferior area compared to other companies?

Murata [A]: First, it was finally determined that the QTc interval prolongation that occurred was not a grade three or higher event and was not related to enzomenib. However, the event of QTc interval prolongation itself has actually been confirmed, and we have reported this in our presentation. However, we do not believe that QTc interval prolongation is significantly increased by enzomenib.

Wakao [Q]: I understand. I understand that you continue to anticipate that this may be the best-in-class in terms of safety.

Secondly, I would like to know about nuvisertib. The monotherapy data does not tell us much about the efficacy of monotherapy because the time points for nuvisertib are difficult to compare to data for other drugs. On the other hand, with regard to the combined use, I can see the sense in using them together, because I can see a strong kind of add-on effect.

Is this drug not so much best-in-class in monotherapy, but is it a drug whose mechanism is different from others, so that its value becomes apparent when used in combination?

Murata [A]: I took your question to mean how we evaluate the results of the single agent. Since we are testing on relapsed/refractory patients, we by no means believe that it is the improvement of total symptoms or the reduction of the spleen that is bad. We are very excited about the potential of this drug as a single agent, or rather, we believe it has solid potential.

To illustrate its position as a development strategy, we have introduced today its use in combination with momelotinib. We believe that this is a chance, as we accept that the added benefit of combining the two products and the combined effect of the two products is being recognized.

There continues to be an unmet medical need for patients who have relapsed, even with a single agent, or who have used ruxolitinib as well as other JAK inhibitors and no longer have treatment options. We are interested in the possibility of having it used as a single agent in such places, and this is also under consideration at this time.

Wakao [Q]: I understand.

I'm not sure about the monotherapy data because the time points are different from the others. Does your company feel that monotherapy is showing solid results and that data is accumulating to show that monotherapy can compete adequately?

Murata [A]: Yes. That is correct.

Wakao [Q]: I understand. Thank you very much.

Another thing is that you are now accumulating more data to increase the value of your partnership activities. What kind of data, if accumulated, would contribute to increasing the value of each drug?

Since data on the combination with enzomenib is still scarce, I think it will become more valuable as data on the combination accumulates and can demonstrate the potential of the first line. If anything, nuvisertib would be more valuable as a combination drug if more data were accumulated on its use in combination with momelotinib. I think that it is important to accumulate such data.

Sato [A]: You are almost right. Regarding enzomenib, we cannot share the results of the Phase II study with KMT2A as a single agent until the results are finalized. We believe that it is the time when the results will be compiled or when the data for the combination of first-ever AML will be available.

In the case of nuvisertib, as you mentioned, I think it is the time to accumulate more data on the combination with momelotinib, or to decide on or start the specific design of the Phase III trial.

Wakao [Q]: I understand.

I would like to know one more thing. I feel that this drug works well for KMT2A. Is this easy to work for it mechanistically? I believe the other drugs did not respond well to KMT2A. What is the reason for the good response of this drug?

Murata [A]: The research team has been working on the issue of what is mechanistically different. As you mentioned, we believe that the CR rate for patients with KMT2A rearrangements is very high compared to the results of drugs of other companies.

One is that it is being administered well, probably due to tolerability issues, and so on. Specifically in terms of pharmacological mechanism, for example, it is not yet known where in the KMT2A molecule it attaches to so that this works well.

Wakao [M]: I understand. Thank you very much. That's all from me.

Kino [M]: Thank you very much.

Mr. Hashiguchi of Daiwa Securities, please proceed.

Hashiguchi [Q]: I am Hashiguchi of Daiwa Securities. Thank you.

Ms. Sato and Mr. Murata, I would like to ask each of you a question from the perspective of what pace and how substantial we can expect the pipeline to become in the future.

How does the current state of management, which constrains to some extent the resources that can be devoted to each project, affect the state of this pipeline and project efforts? If such restrictions are eased in the future as sales and profits increase, I would like to know what kind of initiatives you would like to increase in the future.

In his presentation, Mr. Sato mentioned that there is a large early-stage pipeline but no late-stage ones. If development progresses smoothly in the future, what kind of projects would you like to increase? If you proceed to the later stages of the project, you could license out it completely and move on to the next project. On page 16, there is only a small mention of commercialization by partners, which I felt was not given much

emphasis. Is it possible to further increase the pipeline by advancing items at the later stages when items come up from the preclinical stage? I would be interested to hear from you in terms of resources and how many projects are accumulating.

Maintenance is very important, especially for enzomenib, considering the symptoms. What I would like to ask Mr. Murata is when and how you will start this development. I think that the combination with Venetoclax/Azacitidine alone may not always meet the needs of patients with different backgrounds and treatment histories. I would like to hear more about the pace at which you intend to expand the development of concomitant use with other existing drugs.

Sato [Q]: Thank you for your explanation. I will answer your question.

As you know, we have made significant reductions in R&D expenditures in FY2023, FY2024, and FY2025. In FY2026, we plan to increase R&D expenditures to a certain extent compared to the current fiscal year, but this does not mean that there will be an abundance of funds.

What will this look like in the future? In the field of CNS, as well as in oncology, there are a lot of pipelines in the late stages of research, and several are being developed that will be ready for clinical trials in FY2026 and FY2027. We want to do as much as possible to move them into the clinic and advance their initial development. We would like to consider the following process for those of these that have been confirmed safe in early development and for which patient signals have been obtained.

Another question was asked if we are licensing out. We have not made a definite decision on that at this time. First of all, as for the CNS in particular, we will have to confirm certain safety and efficacy signals before we can consider what to do.

Of course out-licensing is an option, but I believe that we have all kinds of options, including joint development and proceeding on our own. The decision will be made through internal consultation, taking into consideration factors such as our overall pipeline situation and development budget at the time.

Murata [A]: I think the first question about enzomenib is how to develop maintenance.

We have patients who are currently participating in Phase I of our relapsed/refractory trials, dose escalation trials and dose optimization trials, who are being transitioned to transplantation. This is a patient who reached CR after treatment was completed and was transitioned to transplantation when the opportunity arose. Some of those patients continue to cooperate with us in the administration of enzomenib. In this context, the study of how to set the dosage during maintenance has already started now.

In addition, many academic professors are very interested in our work, as a result of our presentations at the American Society of Hematology and the European Society of Hematology last year, and we have been approached by such professors. We will also work to accumulate evidence while using that network of academia.

We have also partnered with the National Cancer Institute in the US, and are working with US research institutions to find out what kind of diseases enzomenib can be used for. We expect that a very variety of opportunities will emerge in the future.

Hashiguchi [Q]: Thank you very much.

Do you have a similar approach to the development of combination therapies other than the combination with Venetoclax/Azacitidine?

Murata [A]: Yes, we do. Specifically, we have received requests from several professors to work with us on combination studies of certain agents with enzomenib. We feel that we are getting a good response from people who are interested in our products. We would like to use such a network in combination with other than Venetoclax/Azacitidine to successfully accumulate evidence and expand opportunities for future development.

Hashiguchi [M]: I understand very well. Thank you very much. That's all from me.

Kino [M]: Thank you very much.

Mr. Wada of SMBC Nikko Securities, please proceed.

Wada [Q]: This is Wada from SMBC Nikko Securities. Thank you very much.

I would like to ask about the platform nature of SMP-3124 for liposome formulations and the possibility of horizontal development. I know that there are already several drugs approved for liposomes, but is there some aspect of your liposome technology that differentiates it from the technology of other companies?

I am aware that you are now doing Phase I/II for various types of cancer. I would like to ask about the possibility of having to change the design for each cancer type, or about possible bottlenecks in horizontal development.

Sato [A]: Thank you for your question.

The first point is about the uniqueness of our company as we increase our pipeline in the future. Indeed, we also use existing liposomal nanomedicines. We have a great deal of expertise in the area of how to make liposomes when making individual items, and we believe that our technology is very unique.

For SMP-3124, for example, there are no plans at this time to change the design for each cancer type.

Wada [Q]: Regarding this Phase I/II data, the initial data on safety and other aspects will be available in December 2028, but is there any possibility that the results will be available earlier than that?

Sato [A]: We will be presenting the data a little before 2028, in a conference presentation. However, we will be presenting at another solid tumor conference, not at a hematology conference. There is not one that has been adopted and decided at this time.

Wada [M]: I understand. Thank you very much. That's all from me.

Sato [M]: Thank you very much.

Kino [M]: Thank you very much.

There being no other questions, we will conclude the Q&A session with analysts and investors.

Analysts and investors are free to leave the room as the Q&A session with the press will follow.

Thank you for your patience. Next, we would like to turn to the question-and-answer session from the press. The Q&A session will be held until 15:40.

First, I would like to ask the members of the press present at the Osaka headquarters to raise their hands if they have any questions.

Tomiyama [Q]: This is Tomiyama from Yomiuri Shimbun. I would like to ask you three questions related to AMCHEPRY, which President Kimura mentioned at the beginning of this presentation.

Managing Executive Officer Sato explained that the practical application in FY2025, which is mentioned in the document, is to obtain approval with conditions and deadlines. I am sure we will be able to confirm this again based on the results of the 19th. What are your current goals for the market launch?

Kimura [A]: Thank you for your question.

I can't talk too much about the future, since only the schedule for the review was just announced, but we are confident that the approval will go smoothly as we expect, and that it will probably be conditional and time-limited.

The definition of "market launch" is difficult to define, but we consider it to be when its NHI price is determined. In that sense, I believe we can do it in H1 of FY2026, which starts in April. We can't control that ourselves.

Tomiyama [Q]: Do you mean around H1?

Kimura [A]: It is between 1Q and 2Q. It is our internal outlook.

Tomiyama [Q]: The second point is also a question based on the assumption that if approved. What do you envision the impact and tailwind on the FDA's review in the US if you receive approval from the Japanese regulatory authorities with conditions and deadlines?

Kimura [A]: The clinical trial has started and is running now, and we have to wait the review in the US a little more. I think the conditional and term-limited approval itself will provide psychological support, but more than that, the clinical data will probably build up, including safety data, and they will understand that.

However, the clinical usage itself or the details are not the same in the US and Japan. So I think it will be treated as reference data. We are going to do our best to explain this to the US authorities.

Tomiyama [Q]: On the third and final point, I think the name of the product AMCHEPRY is unfamiliar to me, or perhaps a coined term. What is the origin of this name?

Kimura [A]: As I explained at the beginning, we understood that the product name is also subject to the approval review, so until now we have only disclosed the generic name. We released the name of the product we envisioned in a hurry on Friday seeing the situation.

As you say, this is a coined term. There is an English word "ameliorate," which means to improve. There is an Egyptian sun god named Khepri, which means rebirth, or the rebirth of the sun. We decided to combine these two words to name our drug AMCHEPRY because it is appropriate for our drug, the dopamine progenitor cell mechanism we are aiming for, and the expected efficacy of our drug.

Tomiyama [M]: I understand. Thank you very much.

Kino [M]: Thank you very much.

The next person, please.

Shimizu [Q]: Thank you. My name is Shimizu from Sankei Shimbun.

I too would like to ask in relation to AMCHEPRY. I believe that your company's regenerative and cellular business is targeting sales revenue of approximately JPY350 billion in the late 2030s. If the drug is approved, how do you plan to ensure stable production and spread the drug? I believe that the situation will be different from the case of oral medicines. Please tell us how you plan to achieve the approximately JPY350 billion.

Kimura [A]: We are now working on a project for ophthalmology products as well as Parkinson's. This JPY350 billion is an accumulation of such things, and the figure includes not only Japan but also the United States.

In promoting the use of the product, there is first of all a supply-side problem. Can we supply enough? Especially for Parkinson's, we are ahead of our competitors and the technology is well established. We still need to prepare the equipment and are gradually adding more at S-RACMO, but there are no technical concerns.

On the other hand, since it is a surgical procedure, we need to explore a little more about how to transfer it to medical institutions. The technology is established, but spreading that same technology to Japan and the US is quite different from promoting ordinary pharmaceutical products.

Shimizu [M]: Thank you very much.

Kino [M]: The person in the front seat, please.

Misumi [Q]: I am Misumi from Nikkei Shimbun. Thank you very much.

I am sorry that our questions are concentrated to AMCHEPRY, but first, and I don't know if you can answer this, but can you tell us how confident you are for approval?

Kimura [A]: We have experience with various approval applications. It would be presumptuous to say that we are confident, but we did the best we could. Now we are just waiting for the results of the subcommittee's discussions. Of course we are confident, but we don't know. Now we are waiting quietly.

Misumi [Q]: I understand. Thank you very much.

I believe that there have been a number of products that have received conditional approval but did not receive full approval. In order to dispel such things, I think it will be necessary to accumulate post-sales data and collect good data. Also, hypothetically speaking, if you were to receive a conditional and time-limited approval, could you tell us what you would do to achieve this?

Kimura [A]: we do not know the real reason for the results so far. We believe it is necessary to conduct a Phase IV trial, which is similar to a clinical trial, with the cooperation of hospitals and patients in order to obtain solid data, and we are steadily making preparations for this.

Misumi [Q]: Thank you.

Finally, we recently received news that Takeda has ended its program with CiRA after 10 years. I thought this was headwind news for iPS therapy. Please let me know how you take this.

Kimura [A]: In terms of the whole effort around iPS, that was a bit disappointing news. However, two drugs of ours and Qualipse's are now in the process of being reviewed for approval. If these are successfully approved, I think it will be a big boost.

I am not too surprised about the Takeda case, as we have been talking about it for some time now.

Misumi [M]: That's all. Thank you very much.

Kino [M]: The next person, please.

Okada [Q]: My name is Okada from YAKUJINIPPO.

First, I will ask about the two cancer products. As for nuvisertib, you will be working on Phase III, and as for enzomenib, you will accelerate Phase II, which is currently being implemented. Do you have already accumulated some data on enzomenib?

Kimura [A]: Murata will explain. One thing that was left out in the previous explanation is that this is Phase II, but this is a pivotal study, and we can apply for approval with it. I would like to add that explanation was left out.

Murata [A]: As Kimura just added, the FDA has issued guidelines for acute myeloid leukemia. In this, a path is allowed for certain populations with particularly poor prognosis and relapsed/refractory patients, where the results of the Phase II trial will be used to apply for approval.

Therefore, we are aiming to file an application in the US with the results of the Phase II trial.

Okada [Q]: Could you give us an overview of the nuvisertib Phase III trial, including the region and whether it is a single agent or a combination, as far as you can tell at this point?

Murata [A]: We are actually already working on various things for the Phase III trial. Although it is a Phase I or II trial, it has now started in a very large number of facilities in various countries. We would like to have those facilities involved from the Phase I and II stages to lubricate Phase III, which will proceed later. It is also very important to familiarize them with nuvisertib administration and clinical trials. So we have been starting up for a year or two now, and we are actually working with them on Phase I and II trials.

One idea for the design of the Phase III trial is to use it in combination with momelotinib, which I mentioned earlier. One more thing, as discussed, we are continuing to look into the possibility of single agents. We hope to proceed with a good combination of these trials.

Okada [Q]: Last question. Regarding the whole thing, on page 16, after the launch of the two oncology products, there is the launch of the CNS pipeline. Do you envision the launch of CNS next to iPS cells and cancer?

Sato [A]: We wrote the second half symbolically. We have written three CNS pipelines after enzomenib and nuvisertib are scheduled for launch in 2027 and 2028. We believe that depending on progress, the approval and launch date of SMP-3124 may be even earlier. We believe this depends on which data we are gathering we proceed with.

Okada [M]: I understand. Thank you very much.

Ishii [M]: I am Ishii from Iyakukeizai.

What are your prospects for expanding SMP-3124 to the types of cancers with unmet medical needs that you mentioned?

Sato [A]: The first place we are thinking about from the creation process and the data we are getting is platinum-resistant ovarian cancer. The Phase I trial is designed to cover a wide range of solid tumors, and I believe that we will proceed with the cancer types for which the efficacy can be confirmed by actual data.

Ishii [Q]: Do you have any specific candidates as of yet?

Sato [A]: Other than that, we are still considering. Originally, there were a number of prior products for this CHK1 inhibitor. Although the mechanism was expected to be effective, unfortunately, they have not been developed successfully due to safety issues. We believe there is a wide range of potential.

Ishii [M]: I understand.

Kino [M]: The person in the front, please.

Hashimoto [Q]: My name is Hashimoto from Nikkei BP.

I am not talking about individual items, but would like to ask about the research structure. Over the past few years, there has been considerable downsizing and reorganization in the United States. Are overseas research sites still maintained at the same size or have they shrunk considerably?

Sato [A]: The restructuring process in North America is quite complicated. 7 companies were reduced to one in the summer of 2023. At that time, we decided to do some of the things we were doing as they were, although we restructured some of them, so we had a large number of employees. Then, when the US operations were largely reorganized at the end of FY2023, we downsized the late-stage development team, primarily in CNS.

Meanwhile, the organization in charge of oncology is consolidating as progress is made on these two products.

In the US, there are activities for products such as Rhythmic, GEMTESA, etc., so there is a certain level of organization.

Hashimoto [Q]: I think there were quite a few Sepracor-derived developments, but have you stopped all of them?

Sato [A]: Sepracor, or ulotaront, the late CNS that was done at the former Sunovion, was licensed to Otsuka, and the development of SEP-4199, which was at a late stage, was discontinued.

We have stopped the items of Phase I at that time and are now in the process of developing the next strategy by dividing them into tier one, 02, and 03, as I explained earlier.

Hashimoto [Q]: Although there were many items that were discontinued, you mentioned earlier that more early items are to come. Do you have no idea about reviving something that had been discontinued?

Sato [A]: We do have some suspended items that will be revived. Also, we are thinking of advancing what has been created mainly by Japanese research organizations.

Hashimoto [Q]: Is what is about to emerge originated in Japanese research organizations? Are there not many resources left for foreign ones?

Sato [A]: We have a CNS development organization, but our research organization is mostly downsized. So I think they will come up from Japan.

Hashimoto [M]: Thank you.

Kino [M]: Thank you very much.

Due to time constraints, we would like to ask each of you to limit your questions from this point on to one question.

The next person, please.

Sakaguchi [Q]: My name is Sakaguchi from Iyakukeizaisha.

Regarding AMCHEPRY, how large do you actually expect its sales to be in the future?

Kimura [A]: At this point in time, we have about 4 patients, but the number of patients multiplied by the drug price is the scale of sales.

The amount of sales will not be disclosed. There are 300,000 patients in Japan. In the United States, the number of patients is 700,000 or 1 million. This is a kind of transplant medicine. It depends on how many of them are applicable.

We expect that, given the effectiveness and invasiveness of the treatment, it will be applicable to a significant number of people. I believe we can make sales of over JPY1 billion on a global basis.

Sakaguchi [Q]: Are you saying that you expect the product to be as good as ORGOVYX?

Kimura [A]: ORGOVYX may be a bit larger, I cannot say. We want to make our products available to as many people as possible.

Sakaguchi [M]: Thank you very much.

Kino [M]: The next person, please.

Kinjo [Q]: I'm Kinjo from NHK.

I would like also to ask about AMCHEPRY. You mentioned earlier about post-marketing surveillance, like Phase IV, like a clinical trial. How many cases would the protocol consist of, and would it be done as a single or double? I believe those will naturally be included in the agenda of review. You may not be able to be more specific at this point, but what do you envision? Do you have plans to announce that at some point?

Kimura [A]: There is a post-marketing surveillance for any ordinary drug, but in the case of approval with conditions and time limits, a study to prove efficacy is required, so I think we will conduct something almost like a clinical trial. We will not know what kind of patients it will target and how large it will be until it is approved. So I will refrain from answering for now.

Kinjo [M]: I understand. Thank you very much.

Kino [M]: The next person, please.

Kuriyama [Q]: I am Kuriyama from YAKUJINIPPO.

I too would like to ask about AMCHEPRY. I understand that the review process in Japan is about to begin, and since this is a new area of medicine, there may be many points to consider in the deliberation process. What do you anticipate will be the focus of deliberations on the part of the applicant? Could you tell us what you think as the application side?

Kimura [A]: AMCHEPRY is what our industry calls a disease modifier. In short, it is a drug that treats the disease itself, not merely alleviates symptoms or stops the progression of the disease. What kind of index to use to evaluate its efficacy is totally different from that of ordinary Parkinson's disease drugs. That is one point. This is, of course, a cellular product, so what are the possibilities regarding their quality control and others? Also,

it will depend on what kind of guidelines the authorities come up with for clinical settings that involve surgical procedures and need to be set up at the hospital side.

Kuriyama [M]: Thank you.

Kino [M]: I would like to move to the web.

Mr. Yokoyama, please.

Yokoyama [Q]: I would like to ask about enzomenib. Last year's presentation at ASH showed data on the combination of Ven/Aza for relapsed/refractory patients. However, in the presentation, data from untreated patients on Venetoclax and menin inhibitors were given, and I understood that this was designed for the patients with first-episode. However, in the recent announcement, only about one-third of the respondents were over 65 years old, and only one Asian. I understood that you would still have to wait for the data. In your future development, do you intend to focus on unfit patients, or on those who are not suitable for strong chemotherapy, or who are not transplantable, and do you intend to use it in combination with Ven/Aza in first-episode patients? What is your design?

Murata [A]: Thank you for your question.

The design will be developed in consultation with the FDA and PMDA authorities, with Phase III trials envisioned and input obtained. For now, we assume that unfit patients, for whom Venetoclax/Azacitidine is indicated, will be the first eligible patients.

Yokoyama [Q]: What about age? AML is generally a disease of the elderly, but this time only one-third of the patients were included. I became curious about that.

Murata [A]: Since this one is for relapsed/refractory patients, the age group inevitably does not overlap with the age group of so-called first-episode patients. How to set the age range for first-time cases is exactly the point of discussion, and we are discussing this with the authorities.

Yokoyama [M]: I understand. Thank you very much.

Kino [M]: Thank you very much.

Since there seem to be no other questions, we will now conclude the question-and-answer session. This concludes the R&D briefing. Thank you very much for joining us today.

[END]