COMPANY RESEARCH AND ANALYSIS REPORT

Chordia Therapeutics Inc.

190A

Tokyo Stock Exchange Growth Market

12-Dec.-2025

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Summary

Aiming to submit application for approval of rogocekib, a treatment for hematologic malignancies in the second half of 2028

Chordia Therapeutics Inc. <190A> (hereafter, also "the Company") is a bio-venture specializing in small-molecule anti-cancer drug development established as a spin-out by drug discovery researchers from Takeda Pharmaceutical <4502>. With drug discovery through clinical research as its core business, the Company aims for monetization early on by handling manufacturing and sales in-house through strategic partnerships in Japan and out-licensing overseas. It listed on the Tokyo Stock Exchange Growth Market in June 2024.

1. Development status and future outlook of CLK inhibitor rogocekib

The Company currently has five development pipelines, with its lead pipeline being the CLK inhibitor rogocekib (hereafter, "rogocekib")*1 (CTX-712). Rogocekib has completed a Phase 1 clinical trial in Japan, obtaining promising results at a level comparable to existing approved drugs. Based on these results, it is currently undergoing a Phase 1/2 clinical trial (currently in the Phase 1 part) in the US for hematologic malignancies (second- and later-line treatment of acute myeloid leukemia (AML)). Rogocekib is a small-molecule compound with a novel mechanism of action whereby excessive stress applied during the mRNA*2 production process leads to kill cancer cells. If development is successful, the Company estimates a potential market size of ¥200.0bn to ¥400.0bn for the AML second-line treatment area alone (calculated based on the drug price and average treatment period, estimating that there will be up to 18,000 prospective patients in Japan, the US, and Europe). Going forward, the number of AML patients is projected to grow, with the potential market size likewise expected to expand. Furthermore, once rogocekib has obtained approval for second-line and later treatment for AML, the Company's plan is to expand its indications to first-line treatment of AML and treatment of other types of cancer.

*1 In November 2024, "rogocekib" was announced as the generic drug name of rogocekib (CTX-712) by the World Health Organization.
*2 RNA stands for ribonucleic acid, a substance needed to make proteins from DNA, a gene. Types of RNA include messenger RNA (mRNA) transcribed from genomic DNA, and transfer RNA (tRNA) used during protein synthesis.

To increase the success probability of clinical trials in the US, the Company decided, in addition to the once-weekly dosage schedule it originally planned for the Phase 1 clinical trial, to conduct a trial with a twice-weekly dosage schedule and an expanded cohort trial based on it. It therefore expects to start a Phase 2 clinical trial scheduled to be conducted in Japan and the US in 2027. The total number of patients will also be increased from 170, as originally planned, to 225. The Company aims to complete the clinical trials quickly by increasing the number of sites and apply for manufacturing and marketing approval in Japan in the second half of 2028. In the US, it intends to start ramping up licensing activities based on the interim results from the Phase 1 part, which it plans to announce in the mid-2026. If it succeeds in developing rogocekib for those indications, it aims to maximize the product's value by expanding its indications to first-line treatment for AML and treatment of other types of cancer.

2. Results trends

As for FY8/25 results, no business revenue was recorded, and there was ordinary loss of ¥1,769mn (loss of ¥1,824mn in the previous fiscal year). This was mainly due to recording research and development expenses of ¥1,425mn, which centered on clinical trial expenses for rogocekib. In FY8/26, the Company plans to keep pursuing clinical trials of rogocekib as a management priority and continue out-licensing activities for other pipelines while limiting costs. It forecasts research and development expenses of ¥1,590mn and ordinary loss of ¥1,958mn. It had cash and deposits totaling ¥2,548mn at the end of August 2025, but it forecasts that clinical trial expenses for rogocekib will increase further in FY8/27 when it enters the Phase 2 part. It therefore issued stock acquisition rights based on a third-party allotment in September 2025 to secure development funds. Since signing a partner agreement in the US for rogocekib will be possible in FY8/27 at the earliest, it appears that funds will be raised via the stock market for the time being.

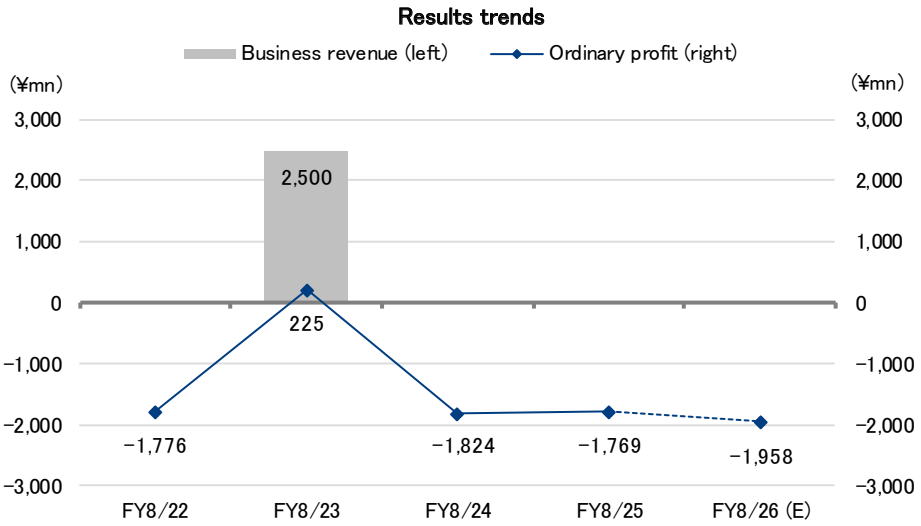
3. Management policies going forward

The Company's vision for 2030 is "To be an R&D-oriented pharmaceutical company based in Japan." It is establishing frameworks for this, such as entering into basic agreements for business partnerships with Shionogi Pharma Co., Ltd. in pharmaceutical manufacturing and MEDIPAL HOLDINGS CORPORATION <7459> in domestic distribution and sales activities in 2022, while undertaking efforts in overseas markets with the release of rogocekib as its top priority, such as aiming for monetization early on through out-licensing. In addition, in April 2025 it was announced that development of CTX-177, which was out-licensed to Ono Pharmaceutical Co., Ltd. <4528> in 2020, had been discontinued and the agreement had been terminated due to the partner revising its development strategy. The Company is now beginning activities to out-license it again. Furthermore, to explore the possibilities of other pipelines as ophthalmological disease therapies, it has begun joint research with Senju Pharmaceutical Co., Ltd. and D. Western Therapeutics Institute, Inc. <4576>. Future developments in this area will be watched closely.

Key Points

- Drug discovery venture specializing in small-molecule anti-cancer drug development established as a spin-out from Takeda Pharmaceutical and listed on the TSE Growth Market in 2024
- Rogocekib currently undergoing Phase 1/2 clinical trial in the US. Plans to announce Phase 1 interim results in 2026 and aims to start Phase 2 in 2027, then apply for marketing approval in the second half of 2028
- For other pipelines, the Company's policy is to pursue early out-licensing and it has also started joint research targeting ophthalmological disease
- In FY8/26, forecasts a somewhat larger loss due to increased development expenses for rogocekib. Funding structure has already been enhanced

Summary



Source: Prepared by FISCO from the Company's financial results and website

Company profile

Spin-out from Takeda Pharmaceutical to pursue small-molecule anti-cancer drug development

1. History

Chordia Therapeutics is a bio-venture established in October 2017 as a spin-out by six drug discovery researchers who had been working on small-molecule anti-cancer drug development at Takeda Pharmaceutical. The six founders selected four of the pipelines they had worked on developing at Takeda Pharmaceutical, entered into a license agreement with Takeda Pharmaceutical in November 2017 for exclusive worldwide rights to research, develop, manufacture, and commercialize them, and commenced development. When Takeda Pharmaceutical was rethinking its overall R&D strategy for the Group around 2016, it decided to streamline in-house development of anti-cancer drugs to new modalities like antibody drugs and cell therapy and place less priority on developing small-molecule compounds. This decision was motivated by a need for business selection and concentration, not because small-molecule compound drug discovery was no longer promising. Against this backdrop, current Chordia Therapeutics CEO Hiroshi Miyake, who was the Japan site head of the oncology drug discovery unit at Takeda Pharmaceutical at the time, joined others to establish a spin-out and continue small-molecule compound drug discovery. The terms of the license agreement with Takeda Pharmaceutical are apparently favorable for the Company, partly reflecting the background to its establishment and because Takeda Pharmaceutical is its principal shareholder with a roughly 15% share.

Rogocekib, which is the lead pipeline, obtained favorable results in a Phase 1 clinical trial begun in Japan in August 2018. The Company therefore started a Phase 1/2 clinical trial in the US with relapsed and refractory AML and myelodysplastic syndrome (MDS)* as the indications in February 2023, and in January 2025, rogocekib was designated as an orphan drug (a drug used for the treatment of rare diseases) by the US Food and Drug Administration (FDA). The benefits of receiving orphan drug designation include waiving of fees when applying for approval, data protection for a maximum of seven years after approval, tax benefits, and streamlining of the approval process. FISCO deems that this will contribute to accelerating review for approval of rogocekib and maximizing its future value.

> * MDS is a disorder wherein normal blood cells (red blood cells, white blood cells, platelets) can be poorly produced due to abnormalities in hematopoietic stem cells from which blood cells in bone marrow develop, and sometimes MDS progresses to AML.

Chordia Therapeutics aims to develop business as a pharmaceutical company in the Japan market. As frameworks for that purpose, it concluded in May 2022 both a basic agreement on collaboration in manufacturing with Shionogi Pharma and a basic agreement on collaboration in distribution and sales promotion with MEDIPAL HOLDINGS. Chordia Therapeutics had 23 employees (including 12 Ph.D.s) as of August 31, 2025, which is an increase of one from the end of the previous fiscal year, and plans to maintain that level for the foreseeable future.

History

Month Year	Main events
October 2017	Established Chordia Therapeutics Inc. in Shonan iPark, Fujisawa City, Kanagawa Prefecture to conduct drug discovery research
November 2017	Entered into a license agreement with Takeda Pharmaceutical Company Limited to acquire exclusive worldwide rights to research, develop, manufacture, and commercialize four pipelines Entered into an investment agreement with Takeda Pharmaceutical Company Limited, Kyoto University Innovation Capital Co., Ltd., and other underwriters
August 2018	Commenced Phase 1 clinical trial for the anti-cancer compound CTX-712 in Japan
March 2019	Entered into an investment agreement with JAFCO Group Co., Ltd., Kyoto University Innovation Capital Co., Ltd., and other underwriters
December 2020	Entered into a license agreement with Ono Pharmaceutical Co., Ltd. to grant it exclusive worldwide rights to research, develop, manufacture, and commercialize the Company's anti-cancer compound CTX-177 and its related compounds
May 2022	Entered into an investment agreement underwritten by Japan Growth Capital Investment Corporation, UTokyo Innovation Platform Co., Ltd., MEDIPAL Innovation Fund, Shinsei Capital Partners, Ltd., Nippon Venture Capital Co., Ltd., and Shionogi Pharma Co., Ltd. Concluded a basic agreement with MEDIPAL HOLDINGS CORPORATION regarding a future business alliance in areas such as distribution and sales promotion Concluded a basic agreement with Shionogi Pharma Co., Ltd. regarding collaboration in manufacturing small-molecule compounds
August 2022	Commenced Phase 1 clinical trial for the anti-cancer compound CTX-177 (ONO-7018) in the US through the licensee Ono Pharmaceutical Co., Ltd.
February 2023	Commenced Phase 1/2 clinical trial for the anti-cancer compound CTX-712 in the US
August 2023	Completed patient enrollment for Phase 1 clinical trial of the anti-cancer compound CTX-712 in Japan
June 2024	Listed on the Tokyo Stock Exchange Growth Market
November 2024	Rogocekib was decided as the generic name of the anti-cancer compound CTX-712
January 2025	The anti-cancer compound CTX-712 received orphan drug designation from the US Food and Drug Administration
April 2025	License agreement with Ono Pharmaceutical Co., Ltd. regarding the anti-cancer compound CTX-177 (ONO-7018) was terminated
July 2025	Started joint research with D. Western Therapeutics Institute, Inc. aimed at developing treatment for ophthalmological disease
August 2025	Started joint research with Senju Pharmaceutical Co., Ltd. aimed at developing treatment for ophthalmological disease

Source: Prepared by FISCO from the Company's prospectus for the issuance of new shares and secondary offering of shares and results briefing materials

Developing cancer therapies targeting RNA deregulation stress

2. Business overview

(1) Management policies

The Company's aim is "Building a world where tomorrow is another day" by developing and delivering ground-breaking new anti-cancer drugs from Japan to patients as soon as possible. Its mission is to develop first-in-class* anti-cancer drugs, and its vision for 2030 is to grow into an R&D-oriented pharmaceutical company based in Japan by achieving that.

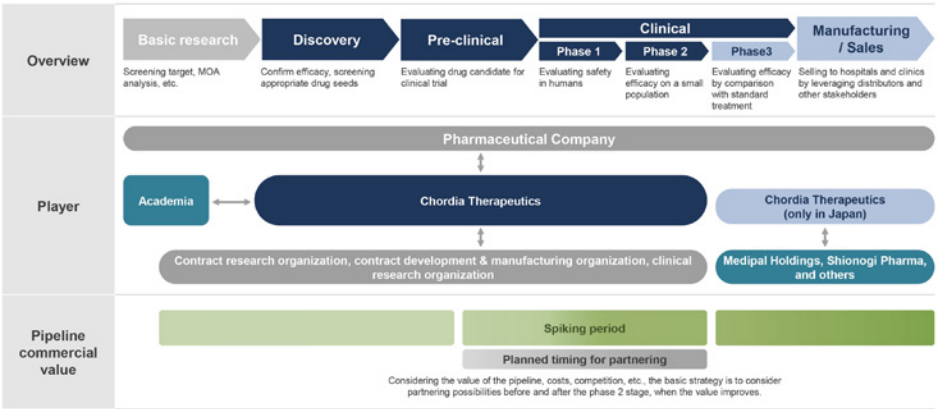
* Innovative pharmaceuticals demonstrating effectiveness differing from existing treatments through a new and unique mechanism of action.

(2) Business model

Chordia Therapeutics' hallmarks are its ability to search for seeds through collaboration with academia and bring drugs to market with drug discovery capabilities cultivated at a pharmaceutical company. It is efficiently advancing collaborative research with academia while utilizing subsidies from the Japan Agency for Medical Research and Development (AMED) as well as working to make development more efficient by utilizing AI technologies and other measures in order to discover biomarkers, which are important in new drug development.

Pharmaceutical development starts with basic research, followed by discovery and pre-clinical research before moving on to clinical trials to confirm safety and efficacy in humans. After that, manufacturing and marketing approval is obtained to bring the drug to market. The Company's basic policy is to position its core business as the part of this process from discovery through Phase 2 clinical trials (evaluation of efficacy in a small number of patients). In addition, it will handle manufacturing and sales in-house in Japan, while utilizing out-licensing in overseas markets. As for the timing of out-licensing, it will consider it around Phase 2 clinical trials when value improves as a basic pattern, reflecting the pipeline's value, costs, and competitive conditions.

Pharmaceutical development process

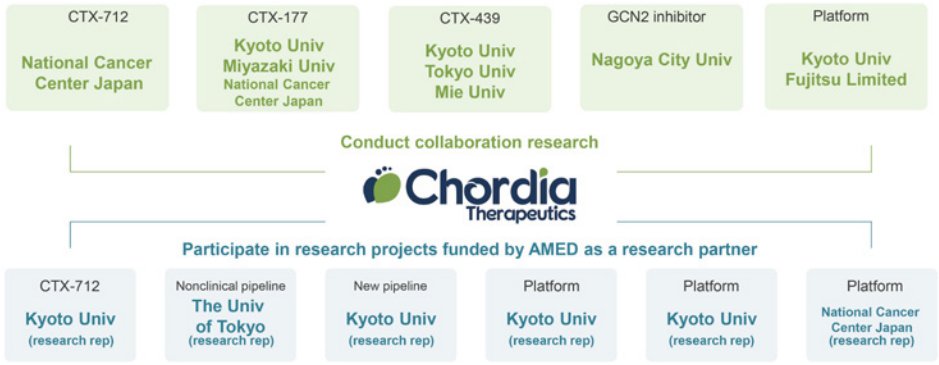


Source: The Company's results briefing materials

Company profile

Chordia Therapeutics is working to discover first-in-class, small-molecule compounds in its target field of oncology where there are strong medical needs. Although predicting safety and efficacy is difficult due to their novel mechanisms of action, these compounds also have the potential to deliver significant therapeutic effects to patients for which existing treatments have been ineffective. Global pharmaceutical companies are also very interested in these compounds since prices for them are often set high according to their efficacy and novelty when calculating drug prices, which is conducive to major license agreements. Chordia Therapeutics is conducting such drug discovery research in collaboration with many academia, and is distinct in that it is proceeding efficiently while utilizing subsidies from AMED on the financial front.

11 collaborative research projects, mainly with academia

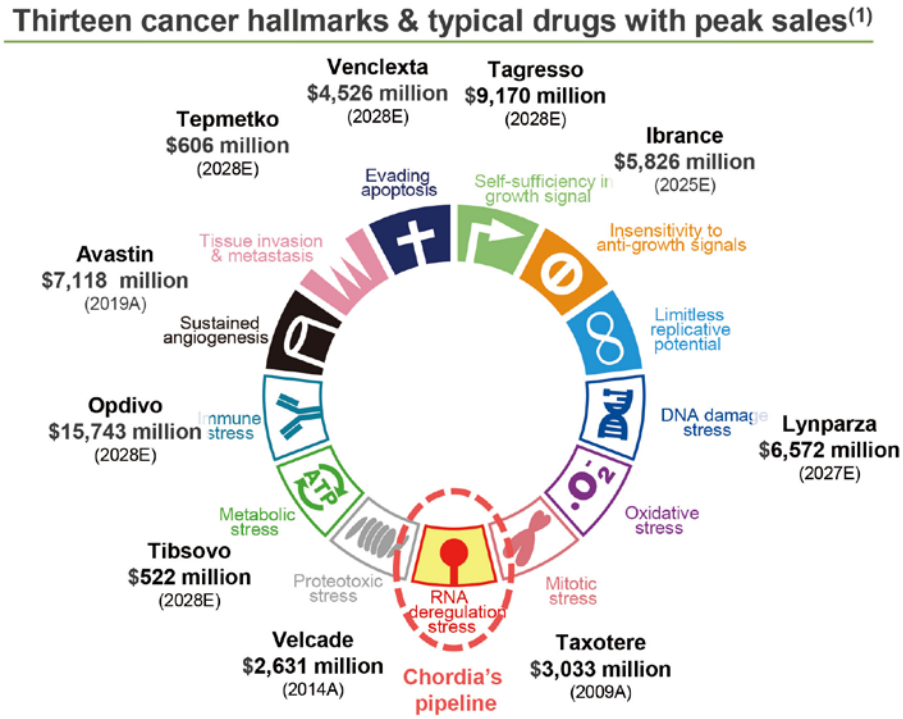


Source: The Company's results briefing materials

(3) Anti-cancer drugs targeting RNA deregulation stress

Discovering hallmarks of cancer and identifying differences from normal cells is considered important for finding molecules for anti-cancer drugs to target. Research in recent years has identified that there are 13 hallmarks in cancer cells, and Ono Pharmaceutical's Opdivo is among numerous blockbuster anti-cancer drugs targeting 10 of those hallmarks, including immune stress and DNA damage stress, that have been developed. Against this backdrop, Chordia Therapeutics is working to develop anti-cancer drugs targeting RNA deregulation stress, which have yet to be launched.

RNA deregulation stress

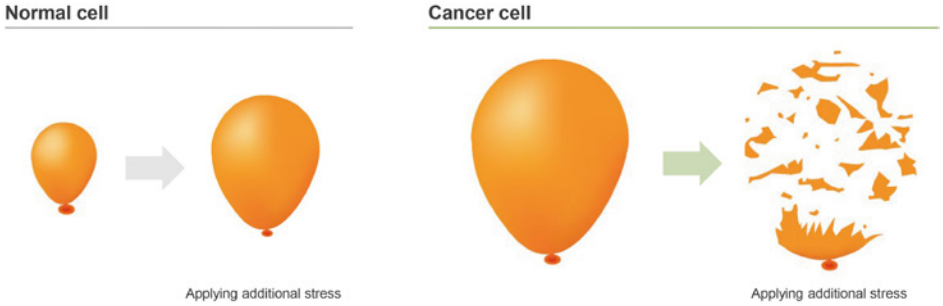


Source: The Company's results briefing materials

Cancer cells are under extreme stress compared to normal cells because of multiple disruptions during RNA generation. Administering anti-cancer drugs that apply even more stress to the overstressed cancer cells kills them. Although administering anti-cancer drugs also puts some stress on unstressed normal cells and produces modest effects*, research has shown that they return to normal as the stress is relieved over time.

* In the Phase 1 clinical trial of CTX-712, side effects such as nausea and vomiting were observed but were controllable with the administration of antiemetic agents, and the safety profile was acceptable.

Concept for anti-cancer drugs

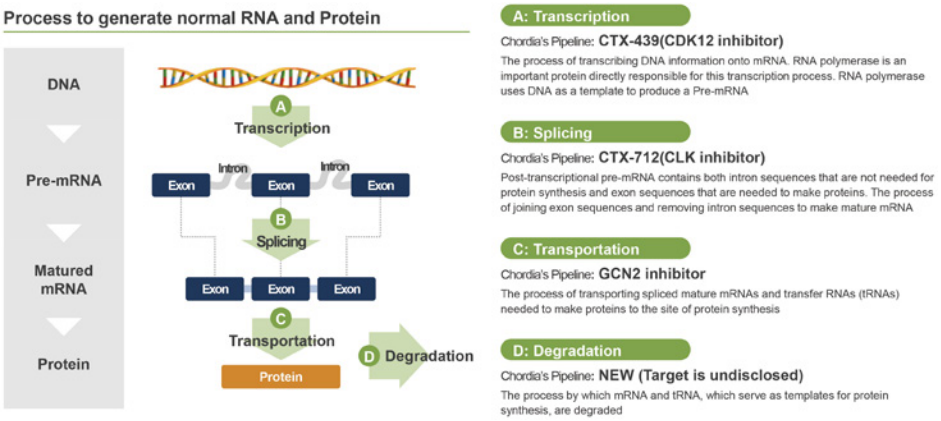


Source: The Company's results briefing materials

Taking a brief look at protein generation in humans, genetic information from DNA is transcribed to generate precursor mRNA, which next undergoes the necessary splicing*1 to become mature mRNA, after which protein is generated by transporting transfer RNA to the site of protein synthesis. The Company is working to develop anti-cancer drugs that inhibit various types of kinase*2 that play a role in processes including transcription, splicing, and transportation.

*1 Process of removing from precursor mRNA the parts that are not necessary (introns) in protein synthesis
*2 Kinase is a general term for enzymes that regulate cell proliferation and other functions.

RNA generation process and Chordia Therapeutics' pipeline modes of action



Source: The Company's results briefing materials

Development pipelines

Expanding cohort for Phase 1/2 clinical trial of rogocekib and aiming to apply for marketing approval in second half of 2028

1. Rogocekib

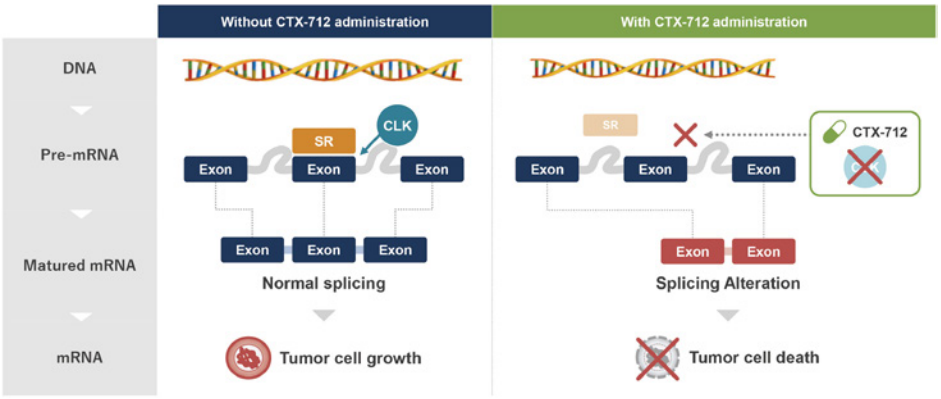
(1) Development status

Rogocekib is currently the lead pipeline. It causes splicing alterations by inhibiting the activity of CLK kinase*, which plays an important role in the mRNA production process. This increases RNA deregulation stress, which is expected to kill cancer cells.

* CLK kinase phosphorylates SR proteins. Phosphorylated SR proteins promote normal splicing by accurately removing unnecessary sections (introns) of pre-mRNA.

Development pipelines

Effects of rogocekib (CTX-712)



Source: The Company's results briefing materials

In a Phase 1 clinical trial in Japan from 2018 to 2023, safety and efficacy were demonstrated in a total of 60 patients lacking effective standard treatment options with AML, MDS, as well as other solid cancers (ovarian cancer, breast cancer, pancreatic cancer, colorectal cancer, sarcoma, etc.). As for the trial design, a dose escalation study with twice-a-week administration* was conducted, and data was collected and evaluated on safety, efficacy to determine maximum tolerated dose, and dose-limiting toxicity as the primary endpoints, as well as pharmacokinetics and other secondary endpoints.

* Dosing is used to restore the condition of normal cells subjected to stress from the administration of CTX-712.

During the course of administration to trial subjects, therapeutic effects were observed in hematologic malignancies, ovarian cancer, and so forth, so enrollment of those patients was prioritized. As a result, 14 patients with hematologic malignancies, 14 patients with ovarian cancer and, 32 patients with other solid cancers were enrolled in the study. Results of the clinical trial were announced at the American Association for Cancer Research Annual Meeting in April 2024. Although adverse events such as nausea, vomiting, and diarrhea were observed, they were controllable with the administration of medication such as antiemetic agents as previously mentioned, and the safety profile was acceptable.

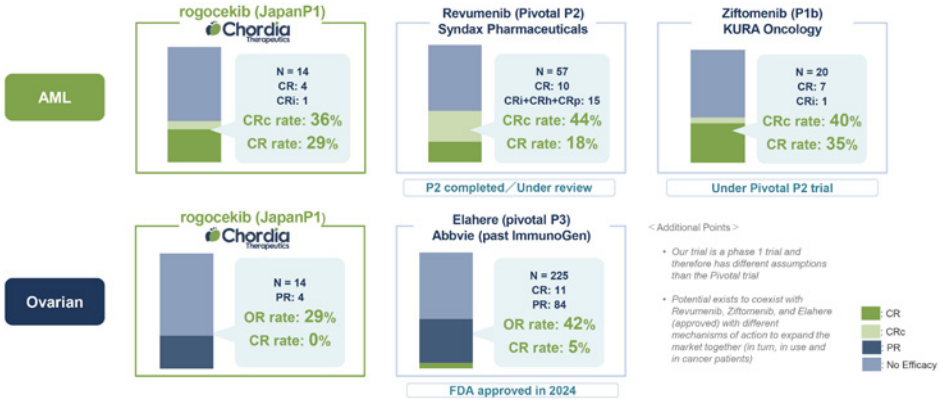
Regarding efficacy, partial response (PR)*1 was achieved in 4 out of 14 ovarian cancer patients, and out of a total of 14 AML and MDS patients (including 12 AML patients), complete remission (CR) was achieved in 4 patients and complete remission with incomplete hematological (neutrophil) recovery (CRi) was achieved in 1 patient. These levels of efficacy for AML and MDS were comparable*2 to Daiichi Sankyo's <4568> VANFLYTA, which was approved as an AML drug. Furthermore, the Company deems that the results are on a par with the results for Phase 2 clinical trials of Syndax Pharmaceuticals' <SNDX> Revumenib (out of a total of 57 patients, CR was achieved in 10 patients and CRi, CRh, or CRp, in which cancer cells are temporarily eliminated from the body, was achieved in 15 patients), which was approved as an AML drug in November 2024, and the results for products being developed by other companies that are nearing the approval application stage.

*1 Partial response (PR) denotes a 30% or greater reduction in the tumor diameter compared to before treatment. In leukemia, complete remission (CR) denotes that the bone marrow contains less than 5% leukemic cells and the number of normal neutrophils and platelets has been completely restored. Complete remission with incomplete hematological (neutrophil) recovery (CRi) denotes the bone marrow contains less than 5% leukemic cells, but neutrophils and/or platelets have not fully recovered.

*2 For VANFLYTA, none of 16 patients achieved CR but 56% of them achieved some kind of response (overall response rate 56%).

Development pipelines

Results of Japan Phase 1 trial of small-molecule anti-cancer drug rogocekib



Source: The Company's results briefing materials

Among 14 subjects with AML and MDS, 3 of the 4 subjects with splicing factor abnormalities at the pre-treatment stage achieved a response, and the treatment duration was 300 days or longer, demonstrating a long-term response and showing a strong correlation between those patients' splicing abnormalities and therapeutic effects. Looking at the ratio of patients with splicing factor mutations by cancer type, it tends to be relatively high for hematologic malignancies at 10–20% for AML and 40% for MDS, compared to a low 1–2% for lung, breast, and other types of cancer. Moreover, AML has a high rate of relapse as standard treatments prove ineffective and a low five-year survival rate of about 30%, so it is a field where the development of novel therapies is strongly desired. Given this situation, the Company decided to first work on developing rogocekib for indication as second- and later-line treatment for AML and MDS. It began a Phase 1/2 clinical trial in the US in 2023*.

* Phase 1 is also being conducted in the US because a capsule formulation was used in the clinical trial in Japan, whereas a tablet formulation is being used in the clinical trial in the US with a view to marketing.

The Company initially planned to complete the Phase 1 part of the clinical trial in the US around the end of 2024 and announce the interim results in mid-2025, but it changed course*1 to increase enrolment in the Phase 1 study in line with Project Optimus*2 proposed by the FDA in 2021. According to the revised version of the clinical trial program announced in September 2025 based on discussions with the FDA, along with the once-weekly, five-dose (20mg, 40mg, 80mg, 100mg, 140mg) dosage schedule that was initially planned for the dose escalation cohort in the Phase 1 part, it has added a new twice-weekly, three-dose (60mg, 80mg, 100mg) schedule.*3 The total enrolment will be around 40 patients, and since 36 patients were already enrolled at the end of August 2025, it expects to complete it before the end of the year. Furthermore, it will narrow down the dosing and administration regimens to two or three patterns with which favorable results are obtained, then compare and assess them by enrolling 60 to 70 patients in an expanded cohort and decide on the dosage and administration for conducting the Phase 2 clinical trial based on the results. While conducting a clinical trial with an expanded cohort that is larger than initially planned will make the trial period longer, the Company believes that it will further increase the probability of success. At the same time, it will conduct a trial with around 20 patients to examine drug interactions in order to determine suitable doses of rogocekib when using it in combination with treatment for fungal infections, which are liable to affect AML patients. The Company intends to complete these trials by the first half of 2027 and start Phase 2 clinical trials in Japan and the US in mid-2027 (planned number of patients: around 100*4). Due to the revision of the clinical trial program, the total number of subjects will increase from 170, as originally planned, to 225. To complete the clinical trials quickly, the Company intends to increase the number of sites, which is currently six, as needed.

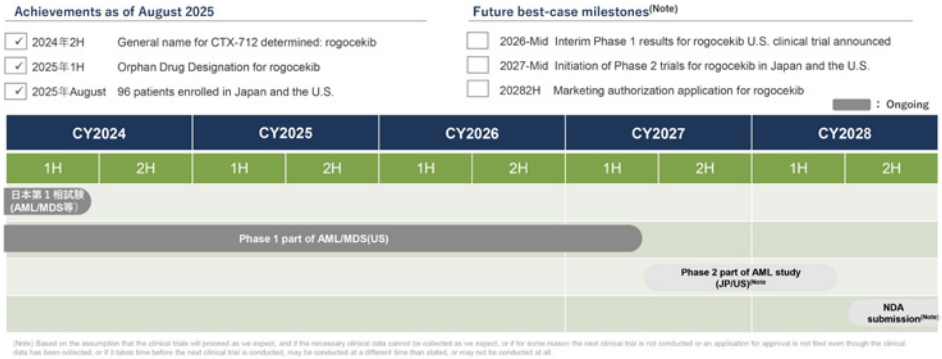
*1 Chordia Therapeutics changed its course based on advice from a consulting company that complying with Project Optimus guidelines is preferable in order to obtain marketing approval after the Phase 2 clinical trial since there have been cases in the US where other companies attempted to submit NDAs for development products not compliant with the guidelines, but they were rejected by the FDA.
*2 For the purpose of optimizing dosage and improving safety in the development of anti-cancer drugs, the FDA provided guidance recommending conducting Phase 2 clinical trials after examining several dosage and administration regimens during Phase 1.
*3 Screening is conducted in 28-day cycles, and if cancer has advanced, the dose is discontinued.
*4 Compared to the 57 patients in Syndax's Revumenib Phase 2 clinical trial, 100 patients is a lot, but this is likely because trials are being conducted in both Japan and the US and the Company has formulated a conservative plan to increase the probability of success.

With the changes to the clinical trial program, the planned schedule for announcing the interim results has also changed from the second half of 2025 to mid-2026. In terms of timing, it seems likely that the results will be announced at a conference on hematological malignancies to be held in June in Europe. Meanwhile, the Company aims to apply for marketing approval in Japan in the second half of 2028 using the SAKIGAKE designation system*. In the US, it will ramp up out-licensing activities based on the interim result data. If the details of the interim results are on a par with the results for existing approved drugs, it may be expected to significantly accelerate the conclusion of agreements. Therefore, there will be focus on the details of the interim results.

* This is system wherein the Ministry of Health, Labour and Welfare (MHLW) designates products in development that meet specified criteria, such as the innovativeness of the medication, the severity of the target disease, very high efficacy for the target disease, and the intent and framework for early development and filing for approval in Japan ahead of other countries (or at the same time), as SAKIGAKE pharmaceuticals, with the aim of early practical application by conducting priority reviews, consultations, and so forth (aims to shorten the review period from the normal one year to six months).

Development pipelines

Development schedule for rogocekib



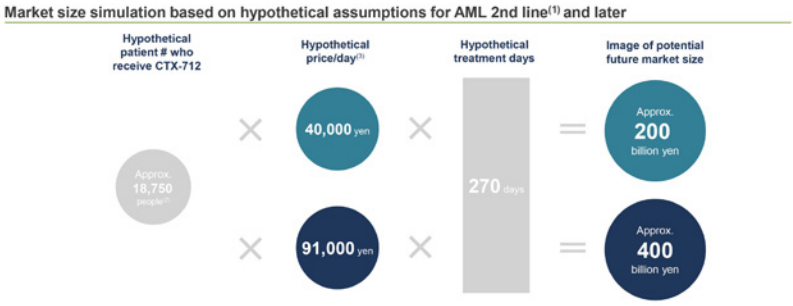
Source: The Company's results briefing materials

(2) Potential market scale as second- and later-line treatment for AML

The number of AML patients is estimated at 50,000 a year in major countries in Japan, the US, and Europe. Some of them do not respond to first-line treatment or relapse, and proceed to second-line treatment. The probability of that is seen as up to 50%, and rogocekib's initial target is patients among them without actionable genetic mutations (FLT3, IDH1/2, etc.) for which treatments are available and patients that move on to third-line treatments after second-line treatments prove ineffective. Chordia Therapeutics estimates the target patient population at up to about 18,000.

Multiplying this prospective number of patients by the price/day referencing existing therapies (¥40,000–¥91,000) and average treatment period (estimated about 270 days) puts the potential market scale at ¥200.0bn–¥400.0bn. The number of AML patients is also trending upward, with the potential market scale expected to expand at the same pace. While there are competitor drugs that have already gone to market, rogocekib could acquire a share exceeding previously released drugs, depending on the results of the clinical trials, and its growth potential may be considered significant. Furthermore, if rogocekib is approved for marketing as a second-line and later treatment for AML, the Company intends to expand its indications to first-line treatment for AML and treatment of other types of cancer, thereby maximizing the product value.

Estimated market scale as second- and later-line treatment for AML



Source: The Company's results briefing materials

(3) Domestic manufacturing and sales system and overseas strategy

The Company plans on in-house manufacturing and sales in Japan, and intends to outsource manufacturing to Shionogi Pharma with which it concluded a collaborative agreement in 2022. As for the US and other overseas markets, the Company plans to develop sales by concluding sales license agreements with global pharmaceutical companies, and has already entered into a non-disclosure agreement with and is providing information to several companies. As mentioned earlier, it plans to start ramping up out-licensing activities based on the interim results of the Phase 1 part, and it could conclude license agreements as early as during FY8/27. The Company intends to conclude license agreements with advantageous terms that maximize product value.

(4) Status of CLK inhibitor competitors

There are two bio-ventures in the US developing CLK inhibitors, but the Company does not view any of them at a threat at this point. Biosplice Therapeutics conducted a Phase 1 clinical trial with single-agent dosing but the overall response rate was 0% (six patients achieved a reduction in tumor size of 10% or more), and at present, investigator-initiated clinical trials are only being conducted for solid cancers such as sarcomas, indicating that efficacy is not being clearly demonstrated. Also, BlossomHill Therapeutics started a Phase 1/1b clinical trial for AML (planned number of patients: 170) in June 2024, which is expected to be completed in 2026. It appears to be about five years behind the Company. Moreover, the drug it is developing is a multikinase inhibitor which inhibits kinases other than CLK, so it is seen as having a higher risk of side effects.

Chordia Therapeutics is in the lead in CLK inhibitor development, and has already registered the substance patent in 51 major countries in the world. Therefore, focus will be on interim results from the Phase 1 part expected to be announced around the middle of 2026, as big deals could follow if clinical trials show good results.

Planning early out-licensing for other pipelines and starting joint research focusing on ophthalmological disease

2. Other pipelines

(1) CTX-177 (MALT1 inhibitor)

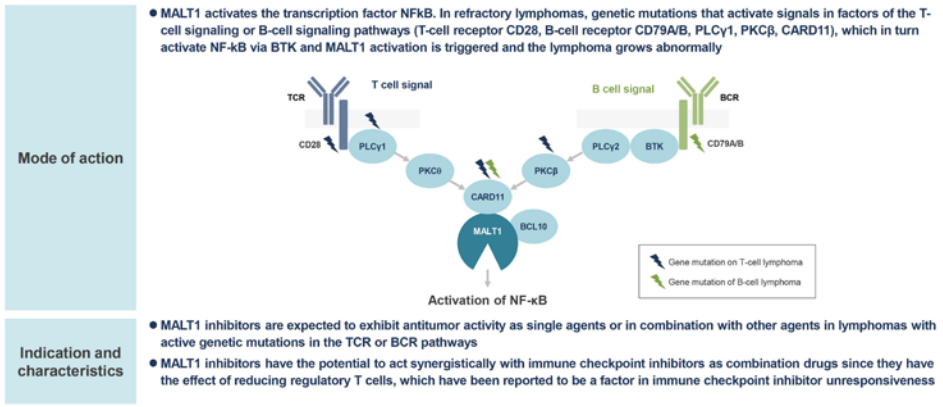
The MALT1 inhibitor is a pipeline being developed as a promising therapy for refractory lymphomas, which the Company out-licensed early on to Ono Pharmaceutical in 2020 after pre-clinical studies*. In August 2022, Ono Pharmaceutical started a Phase 1 clinical trial in the US focusing on relapsed and refractory non-Hodgkin's lymphomas or chronic lymphocytic leukemia (enrolment of first patient in February 2023, target number of patients: 108), as well as starting a Phase 1 clinical trial focusing on relapsed or refractory non-Hodgkin's lymphomas in August 2024 (target number of patients: 24) in Japan. However, in April 2025, it notified the Company that it would cease development of CTX-177 for strategic reasons. As a result, the license agreement was terminated, and all rights reverted to the Company. No financial compensation was received in relation to this, and data from the clinical trials to date was transferred to the Company.

* The Company has already received an upfront payment of ¥0.8bn (FY8/21) and a milestone payment of ¥2.5bn upon the start of Phase 1 clinical trials (FY8/23), and the contract stipulated that it would obtain up to ¥49.6bn for future development and commercialization milestones as well as a percentage of business revenue ranging from the high single digits to low double digits as royalties.

Following the termination of the agreement, the Company began out-licensing activities targeting around 100 companies engaged in developing treatments for lymphomas, and a number of these have shown interest. Since the details of the Phase 1 clinical trial data are not disclosed, it is difficult to predict how future development will unfold, but if it seems that results have been obtained which show the potential for going to market, it is quite possible that CTX-177 can be out-licensed again. The Company is aiming to out-license it during FY8/26.

In refractory lymphomas, it is known that genetic mutations activate signals in factors of the T-cell signaling or B-cell signaling pathways (T-cell receptor CD28, B-cell receptor CD79A/B, PLCγ1, PKCβ, CARD11), and those signals trigger activation of NF-kB via BTK and MALT1 (Mucosa Associated Lymphoid Tissue protein 1), causing the lymphoma to grow abnormally. MALT1 inhibitors are expected to exhibit antitumor activity as single agents or in combination with other agents (BTK inhibitors, etc.) in lymphomas with active genetic mutations in such signaling pathways.

MALT1 inhibitor



Source: The Company's results briefing materials

With regard to the development status of MALT1 inhibitors at other companies, a US bio-venture, Schrodinger <SDGR>, has been conducting a Phase 1 clinical trial (planned number of patients: 52) focusing on relapsed and refractory lymphomas since 2023. It published the early phase clinical trial data in June 2025. Favorable results were obtained: there were no problems with safety and tolerability, and while the complete response (CR) rate for the 45 patients was 0%, the overall response rate including partial response was 22%. In 2026, it is highly likely that Schrodinger will proceed to a Phase 2 clinical trial. For CTX-177, based on the results of pre-clinical studies, the Company expects that it can obtain efficacy results which are at least comparable. Meanwhile, AbbVie (ABBV), which is a major player in lymphoma drugs, has been conducting a Phase 1 clinical trial (planned number of patients: 150) since 2023, which is expected to be completed in 2027. Besides these, Janssen started a Phase 1 clinical trial in the US in 2019, but no new information has been shared about it since then, so it is possible that development has stopped.

(2) CTX-439 (CDK12 inhibitor)

CTX-439 is a CDK12 kinase inhibitor which mainly plays a role in repressing the termination reaction in RNA transcription. When CDK12's function is inhibited, transcription terminates prematurely, short-chain mRNA is produced, and dysfunctional proteins are translated. This is said to have a particularly significant impact on gene clusters involved in DNA damage response. The Company is pursuing development targeting solid cancers with CTX-439 as a single agent or in combination with already approved drugs. In 2024, it acquired pre-clinical data with a view to starting a clinical trial. At present, it is conducting biomarker research aimed at formulating a development strategy and searching for a strategic partner to start a clinical trial.

(3) CRD-099 (GCN2 inhibitor)

CRD-099 is a GCN2 kinase inhibitor. GCN2 is a protein that monitors the intracellular amino acid concentration and is activated when the concentration decreases. When GCN2 function is inhibited, there is no further extracellular uptake and new synthesis of amino acids, and amino acid depletion occurs, inducing cellular death. When used in combination with already approved drugs that lower amino acid concentration, such as asparaginase, it is expected that CRD-099 will have an anti-cancer effect. At present, the Company has completed research to optimize the compound and is at the stage of searching for a strategic partner for conducting pre-clinical studies.

In August 2025, the Company started joint research with Senju Pharmaceutical to explore the possibility of developing GCN2 inhibitors as a treatment for ophthalmological disease. Furthermore, in July 2025, it announced that it will conduct joint research on developing an ophthalmological disease treatment with another small-molecule compound in collaboration with D. Western Therapeutics Institute, Inc.

Development pipelines

Program (target)	Lead indications (cancer type)	Region	Development status	Development and commercialization rights
CTX-712 (CLK)	AML/MDS, ovarian cancer, other solid cancers	Japan	Phase 1 clinical trial completed	In-house
	AML/MDS	US	Phase 1/2 clinical trial underway	In-house (searching for sales licensee)
CTX-177 (MALT1)	Lymphoid malignancies	US and Japan	Phase 1 part of clinical trial underway	In-house (aims to out-license again)
CTX-439 (CDK12)	Solid cancers	-	Pre-clinical study completed	In-house (aims to out-license early on)
CRD-099 (GCN2)	Hematologic malignancies, solid cancers	-	Discovery	In-house (aims to out-license early on)
New pipeline	Hematologic malignancies, solid cancers	-	Discovery	Undecided

Source: Prepared by FISCO from the Company's results briefing materials

Results trends

No business revenue recorded and operating loss comparable to previous fiscal year posted in FY8/25

1. Overview of FY8/25 results

In FY8/25, no business revenue was recorded, and there was operating loss of ¥1,789mn (loss of ¥1,801mn in the previous fiscal year), ordinary loss of ¥1,769mn (loss of ¥1,824mn), and net loss of ¥1,785mn (loss of ¥1,827mn).

Results trends

FY8/25 results

(¥mn)

	FY8/24 Results	FY8/25		YoY Change	Vs. plan Change
		Company plan	Results		
Business revenue	-	-	-	-	-
Research and development expenses	1,499	2,025	1,425	-74	-599
Rogocekib	1,018	1,610	1,070	52	-540
CTX-177	0	0	0	0	0
CTX-439	132	18	27	-105	9
Other (including personnel expenses)	347	396	331	-16	-65
Other administrative expenses	301	408	364	62	-43
Operating income	-1,801	-2,434	-1,789	11	644
Ordinary profit	-1,824	-2,378	-1,769	55	608
Extraordinary profit or loss	-	-	-13	-	-
Net income	-1,827	-2,380	-1,785	41	594

Source: Prepared by FISCO from the Company's financial results and results briefing materials

Looking at the factors behind the changes in business expenses, research and development expenses declined ¥74mn year-on-year (YoY) to ¥1,425mn. As for the breakdown, expenses for rogocekib rose ¥52mn to ¥1,070mn due to the progress of the Phase 1/2 clinical trial in the US, while the Company strove to limit costs for other pipelines, with expenses for CTX-439 decreasing ¥105mn to ¥27mn and other development expenses (including personnel expenses) dropping ¥16mn to ¥331mn. Other administrative expenses increased ¥62mn to ¥364mn, mainly due to higher patent-related expenses. Non-operating income and expenses improved ¥43mn YoY. This was because the ¥28mn in stock listing expenses and ¥8mn in stock delivery expenses recorded in the previous fiscal year were eliminated, while grant income from AMED increased ¥5mn. Furthermore, the loss amount decreased compared to the Company's initial plan, which was mainly because research and development expenses were less than forecast, especially for rogocekib.

Losses expected to grow somewhat in FY8/26 due to increased development expenses for rogocekib

2. FY8/26 forecasts

In FY8/26, the Company also plans to record no business revenue and forecasts a slight increase in losses with operating loss of ¥2,008mn (¥1,789mn loss in the previous fiscal year), ordinary loss of ¥1,958mn (¥1,769mn loss), and net loss of ¥1,960mn (¥1,785mn loss).

FY8/26 results forecasts

(¥mn)

	FY8/25 Results	FY8/26 Company plan	YoY Change
Business revenue	-	-	-
Research and development expenses	1,425	1,590	164
Rogocekib	1,070	1,131	61
CTX-177	0	17	17
CTX-439	27	22	-5
Other (including personnel expenses)	331	420	89
Other administrative expenses	364	418	53
Operating income	-1,789	-2,008	-218
Ordinary profit	-1,769	-1,958	-188
Extraordinary profit or loss	-13	-	-
Net income	-1,785	-1,960	-174

Source: Prepared by FISCO from the Company's financial results and results briefing materials

The Company is planning to increase research and development expenses ¥164mn to ¥1,590mn. In terms of the breakdown, rogocekib expenses will increase ¥61mn to ¥1,131mn due to an increase in clinical trial subjects in the US, while ¥17mn in expenses will be recorded for CTX-177 due to resuming out-licensing activities. Other development expenses are also expected to increase ¥89mn to ¥420mn. On the other hand, the Company plans to reduce CTX-439 research and development expenses ¥5mn to ¥22mn, under the assumption that only activities funded by grant income from AMED will be conducted. It forecasts other administrative expenses, mainly expenses related to registering and certifying patents and insurance expenses, will rise ¥53mn to ¥418mn. We at FISCO believe that the level of research and development expenses could increase around 1.5 to 2 times in FY8/27 due to the acceleration of rogocekib clinical trials.

Issued stock acquisition rights based on a third-party allotment for the purpose of raising development funds for rogocekib

3. Financial position

Looking at the financial position at the end of FY8/25, total assets declined ¥1,951mn from the end of the previous fiscal year to ¥2,681mn. Cash and deposits decreased ¥1,780mn due mainly to expenditures on research and development, while advance payments and prepaid expenses also decreased.

Total liabilities decreased ¥226mn from the end of the previous fiscal year to ¥244mn. This mainly owes to a ¥262mn increase in accounts payable-other. In addition, net assets decreased ¥1,724mn to ¥2,437mn. Retained earnings decreased ¥1,785mn due to the recording of a net loss.

Cash and deposits at the end of FY8/25 were ¥2,548mn, which was close to the Company's target of funds for one year of business activities. Therefore, the Company issued stock acquisition rights based on a third-party allotment in September 2025 (9th to 11th series, equivalent to 17.20 million shares, approx. 25% dilution rate). The 9th series (equivalent to 10.32 million shares) included an exercise price adjustment clause, with ¥105 (initial exercise price: ¥175) set as the minimum exercise price. On the other hand, the exercise prices for the 10th series (equivalent to 3.44 million shares) and 11th series were fixed at ¥175 and ¥210, respectively. If all the stock acquisition rights are issued, the Company will raise ¥2.41bn to ¥3.44bn (minimum exercise price to initial exercise price). In terms of using the funds, the Company plans to allocate them to trial expenses related to the expanded rogocekib cohort and assessment of drug interactions to be conducted by FY8/27. Since clinical trials of rogocekib will continue in FY8/28 as well, it is possible that the Company will conduct equity financing again by then, but it will depend on license agreement negotiations in the US.

Balance sheet

(¥mn)

	End-FY8/22	End-FY8/23	End-FY8/24	End-FY8/25	Change
Current assets	4,482	4,891	4,605	2,669	-1,936
Cash and deposits	4,254	4,799	4,329	2,548	-1,780
Non-current assets	16	17	26	12	-14
Total assets	4,498	4,909	4,632	2,681	-1,951
Total liabilities	221	408	471	244	-226
Interest-bearing debt	-	-	-	-	-
Total net assets	4,277	4,500	4,161	2,437	-1,724
Safety					
Equity ratio	94.5%	91.2%	89.8%	90.8%	1.0pp
Interest-bearing debt ratio	-	-	-	-	

Source: Prepared by FISCO from the Company's financial results and prospectus for the issuance of new shares and secondary offering of shares

Focusing resources on maximizing rogocekib's product value, aiming for early monetization

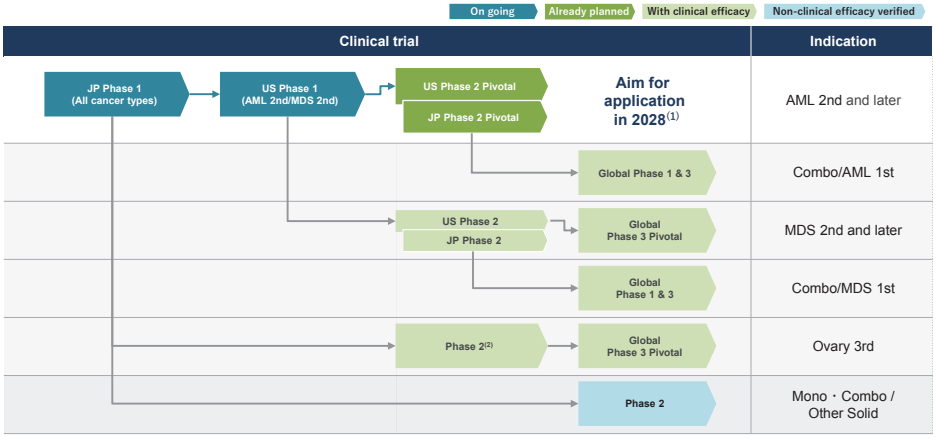
4. Management policies going forward

Guided by its aspiration to develop and deliver groundbreaking new anti-cancer drugs from Japan to patients as soon as possible, the Company is working towards its vision for 2030 to be an R&D-oriented pharmaceutical company based in Japan. Its strategy is to efficiently expand business by handling manufacturing and sales in-house through strategic alliances in Japan and concluding license agreements with global pharmaceutical companies in overseas markets.

Turning to development strategy, the Company's goal for the foreseeable future is to achieve monetization early on by concentrating management resources on rogocekib, working first to obtain marketing approval in the US and Japan for relapsed and refractory AML, then striving to maximize rogocekib's product value by pursuing expansion of indications to first-line treatment for AML, treatment of other types of cancer, and so forth. As previously mentioned, the potential market scale is large and rogocekib has the potential to become a blockbuster, so focus will be on the content of the interim results from the Phase 1 part of the clinical trial expected to be released around mid-2026. FISCO believes the possibility of concluding license agreements overseas will rapidly rise if favorable results are obtained. If rogocekib subsequently proceeds smoothly to the Phase 2 part of the clinical trial and obtains results* on a par with already approved drugs for the CR rate, which is the main evaluation item, it is expected that the Company will aim to submit an NDA in the second half of 2028.

* Syndax Pharmaceuticals' Revumenib, which received marketing approval in the US for the treatment of AML in 2024, had a CR rate of 18% in a Pivotal P2 study. Therefore, the Company believes approval is likely if the CR rate is about 20% (CR rate was 29% in the Phase 1 study conducted in Japan).

Development strategy



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(2) The company anticipates that this portion could become a Pivotal clinical trial in Japan.
Source: Materials provided by the Company

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