

COMPANY RESEARCH AND ANALYSIS REPORT

AnGes, Inc.

4563

Tokyo Stock Exchange Growth Market

14-Oct.-2026

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<https://www.fisco.co.jp>

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Summary

HGF gene therapy product heading toward BLA submission and licensing agreement in 1Q FY12/27

AnGes, Inc. <4563> (hereafter, also “the Company”) is a biotechnology venture originating from the University of Osaka, founded in 1999. The Company’s vision is to become a global leader in gene therapeutics, aiming to deliver innovation for diseases that still lack effective treatments and to contribute to improving the quality of life (QOL) of people worldwide. In 2020, the Company acquired EmendoBio Inc. (hereafter, “EmendoBio”), which develops advanced genome-editing technologies, as a subsidiary. In 2021, it launched testing services through the establishment of AnGes Clinical Research Laboratory (hereafter, “ACRL”), a clinical laboratory in Japan specializing in expanded newborn screening for rare genetic diseases.

1. Status of the HGF gene therapy product

For the HGF gene therapy product, which the Company aims to launch in the US, preparations for a Biologics License Application (hereafter, “BLA”) are underway following the favorable results demonstrated in the late Phase 2 clinical trial. Going forward, the Company plans to submit completed application data sequentially to the US Food and Drug Administration (hereafter, “FDA”) and complete the submission of all data by 1Q FY12/27, with the application expected to be formally accepted as a BLA during the same quarter. As the review period is six months, if the process proceeds smoothly, the Company could obtain marketing approval and commence sales within 2027. The Company is also advancing negotiations on a sales licensing agreement with major pharmaceutical companies, having narrowed the candidates down to around four, and aims to sign a letter of intent (LOI) around December 2026 and conclude a definitive agreement in 1Q FY12/27. Although the Company has received a report from a research firm estimating the number of eligible patients for the HGF gene therapy product in the US at a maximum of approximately 500,000 per year, it estimates the actual number of eligible patients at 200,000–300,000. Assuming the product is administered to 20% of these patients, FISCO estimates that the sales potential could exceed ¥200.0bn.

2. Status of other pipelines

The Phase 2 clinical trial in Japan of NF- κ B decoy oligonucleotide DNA for chronic discogenic low back pain is targeting completion of subject enrollment within 2026 and the announcement of topline data around the end of 2027.

For AV-001, which the Company is jointly developing with Vasomune Therapeutics, Inc. (hereafter, “Vasomune”), subject enrollment in the early Phase 2 clinical trial in North America for acute respiratory distress syndrome (ARDS), including viral and bacterial pneumonia, is scheduled to be completed in 3Q FY12/26, with topline data expected to be announced within the year. In addition, the Company has newly initiated an investigator-initiated clinical trial aimed at preventing brain injury in hemodialysis patients, and is considering the initiation of a clinical trial on resuscitation treatment for patients with severe burns.

Summary

3. Results trends

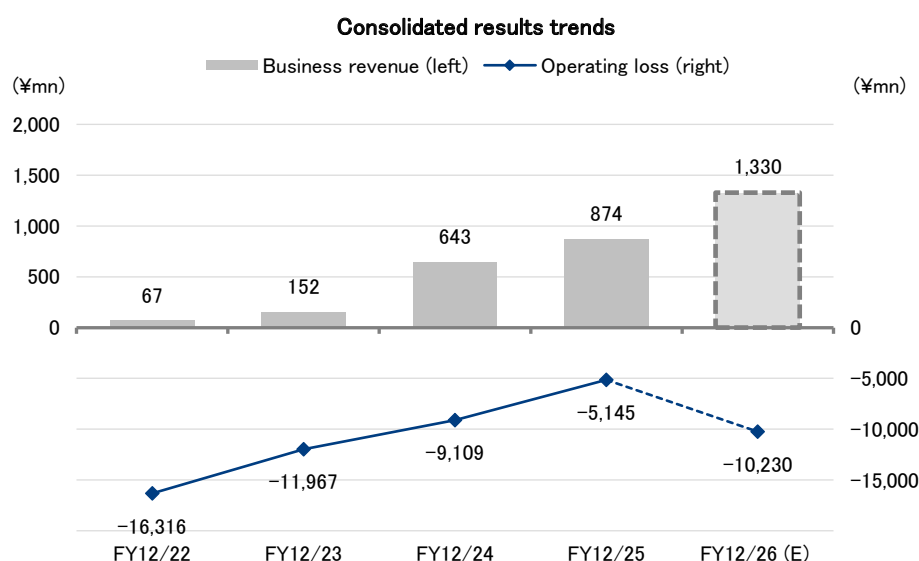
For 1H FY12/26, business revenue was ¥502mn (up ¥87mn year on year (YoY)), while operating loss was ¥3,236mn (widened by ¥835mn). Business revenue increased due to higher sales of Zokinvy, a treatment for progeria. On the cost side, although SG&A expenses decreased by ¥199mn YoY, R&D expenses increased by ¥1,112mn, mainly in relation to the HGF gene therapy product, contributing to the widening of the operating loss.

For FY12/26, the Company has maintained its initial forecasts of business revenue of ¥1,330mn (up ¥456mn YoY) and operating loss of ¥10,230mn (up ¥5,085mn). Business revenue is expected to increase due to growth in sales of Zokinvy, while higher R&D expenses related to the HGF gene therapy product are expected to drive the expansion in loss. If a licensing agreement for the HGF gene therapy product is concluded within 2026, a meaningful upward revision to the forecast could be expected.

Although cash and deposits declined sharply to ¥281mn at the end of 1H FY12/26, in August 2026 the Company issued ¥2.0bn of 2nd series unsecured convertible bonds with stock acquisition rights, as well as the 47th and 48th series of stock acquisition rights, allotted to a fund operated by Growth Partners Co., Ltd., thereby securing funds for its business activities for the time being. If all of the stock acquisition rights are exercised at the initial exercise prices, the Company could raise approximately ¥6.5bn. It is also considering further fundraising thereafter, and plans to raise funds for ACRL's testing business through alliances with other companies, organizational restructuring, and other measures. FISCO believes that the conclusion of a licensing agreement for the HGF gene therapy product would change the stock market's valuation of the Company, and developments in this regard warrant attention.

Key Points

- Aims to conclude a licensing agreement for the HGF gene therapy product in 1Q FY12/27
- Clinical trial results for AV-001, a treatment for ARDS, are expected to be announced within 2026
- Aims to expand the testing business by raising funds through alliances, organizational restructuring, and other measures
- Policy of funding business activities for the time being through equity financing and other means



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

Company profile

Biotechnology venture originating from the University of Osaka, specializing in the development of genetic medicine

1. History

The Company is a biotechnology venture originating from the University of Osaka and was established in 1999 with the aim of commercializing research related to the angiogenic effects of hepatocyte growth factor (HGF) genes. For the HGF gene therapy product, the Company entered into exclusive licensing agreements with Mitsubishi Tanabe Pharma Corporation (now Tanabe Pharma Corporation) covering peripheral vascular disease in the US market in 2012 and the Japanese market in 2015. Subsequently, in March 2019, the Company obtained conditional and time-limited manufacturing and marketing approval in Japan for patients with chronic arterial occlusive disease, and sales commenced through Mitsubishi Tanabe Pharma in September of the same year; however, the post-marketing evaluation required under the conditional approval failed to reproduce the results of the Phase 3 clinical trial. Meanwhile, in light of the favorable results from the late Phase 2 clinical trial conducted in the US, the Company decided to revise its development strategy from a strategic perspective, including temporarily withdrawing its application for full approval in June 2024. As a result, the Company terminated its exclusive licensing agreements with Mitsubishi Tanabe Pharma covering Japan and the US.

Among the Company's other pipelines, NF- κ B decoy oligonucleotide DNA, a nucleic acid medicine, produced favorable results in a late Phase 1 clinical trial initiated in the US in 2018 for chronic discogenic low back pain. Following these results, the Company entered into an agreement with Shionogi & Co., Ltd. <4507> in March 2023 for a Phase 2 clinical trial in Japan, and commenced the trial in October of the same year. The Company also signed a domestic sales agreement in May 2022 with Eiger BioPharmaceuticals, Inc. (hereafter, "Eiger") for Zokinvy, a treatment for Hutchinson-Gilford Progeria Syndrome (HGPS), also known as infantile progeria, and progeroid laminopathies (PDPL)*1. The Company obtained manufacturing and marketing approval in January 2024 and launched sales in May of the same year*2.

*1 HGPS (Hutchinson-Gilford Progeria Syndrome), also known as infantile progeria, is caused by mutations in the LMNA gene and presents with severe growth impairment, progeroid facial features such as alopecia, frontal bossing, and micrognathia, as well as skin atrophy, sclerosis, and joint contractures. Ischemic cardiovascular disease becomes apparent with age and is the leading cause of death among HGPS patients, with the average life expectancy reported to be 14.5 years. PDPL (processing-deficient progeroid laminopathy) is also caused by mutations in the LMNA or ZMPSTE24 genes, resulting in the same or similar symptoms and accelerated aging. The number of HGPS patients worldwide is estimated at approximately 150 to 250, while the number of PDPL patients is currently unknown. HGPS is designated as an intractable disease in Japan, where the number of patients is reported to be only several individuals. Annual sales per patient exceed ¥100mn based on the drug price.

*2 Following Eiger's filing for protection under Chapter 11 of the US Bankruptcy Code in April 2024, the agreement related to Zokinvy was transferred to and will continue with Sentynl Therapeutics, Inc., the successor company to the business.

Under its M&A and alliance strategy, the Company signed a co-development agreement with Vasomune in 2018 and made an investment of ¥273mn in 2023, while continuing development of AV-001 (a Tie2 receptor agonist) as a treatment candidate for ARDS. Additionally in 2018, the Company invested in MyBiotics Pharma Ltd., an Israel-based company focused on microbiome research and development. In 2022, it additionally underwrote ¥74mn in convertible bonds, on which an impairment loss has since been recognized. Furthermore, in 2020, the Company acquired shares of EmendoBio, which develops genome-editing technology, and made it a subsidiary.

Company profile

Apart from its pharmaceutical development business, the Company opened ACRL in April 2021 as a clinical laboratory primarily for screening newborns for rare genetic diseases. Working together with General Incorporated Association Clinical & Research Association for Rare, Intractable Diseases (hereafter, “CRARID”)*, the Company launched contract testing services for optional screening tests.

* Although CRARID ended its contract services for optional screening tests at the end of March 2025, the Company has taken over and continues to provide the services.

2. Business features and business model

A key feature of the Company’s business is that it defines gene therapy products, nucleic acid medicines, and DNA vaccines, which utilize the functions of genes, as genetic medicine, and targets intractable diseases and diseases lacking effective treatments, where clear medical needs exist and which the Company regards as part of its social mission. In addition to internally developed products, the Company also in-licenses development candidates that align with its business strategy from overseas ventures and research institutions, thereby strengthening its development pipeline and diversifying risk.

The Company’s business model centers on research and development (with manufacturing outsourced to external specialized organizations) and entering into co-development agreements and exclusive licensing agreements with major pharmaceutical companies for its development candidates. Revenue is primarily generated through upfront payments, milestone payments based on development progress, and royalty income calculated as a certain percentage of product sales after commercialization. The scale and duration of clinical trials vary depending on the target disease, but it is generally said that Phase 1 through Phase 3 clinical trials take approximately three to seven years. If clinical trial results are favorable, an application for manufacturing and marketing approval is submitted to regulatory authorities. After a review period of approximately one to two years, the product is approved and launched if no issues are identified.

In its testing services for rare genetic diseases, the Company primarily undertakes expanded newborn screening tests*¹ to detect rare genetic diseases in newborns, mainly from clinics in the Tokyo metropolitan area. From 2024, it also began receiving orders through local governments. If a screening result indicates the need for further examination, genetic testing (diagnostic testing)*² is conducted. When patients are diagnosed through screening and follow-up diagnostic testing, starting treatment before the onset of symptoms is expected to help inhibit the progression of symptoms. The diseases covered by the testing service are rare genetic diseases outside the scope of mass screening programs conducted by local governments at public expense (covering 20 diseases), and testing is provided for a fee to individuals who wish to undergo it. Currently, ACRL is able to test for 14 diseases*³, including mucopolysaccharidosis and Fabry disease (in male patients only), and the Company intends to expand the scope of diseases ACRL can test for on an ongoing basis.

*¹ The Company provides fee-based testing services for hereditary diseases that are not included in mass screening programs conducted for all newborns at public expense.

*² A diagnostic test used to determine whether a patient has a specific disease by checking for the presence of genetic mutations that cause the disease, when screening results suggest a possible diagnosis or when symptoms indicate a potential corresponding condition.

*³ The service covers the following diseases: mucopolysaccharidosis (types I, II, IVA, VI, and VII), Pompe disease, Fabry disease, Gaucher disease, Niemann-Pick disease type A/B, Krabbe disease, adrenoleukodystrophy, spinal muscular atrophy, severe combined immunodeficiency, and adenosine deaminase deficiency. In Tokyo, mucopolysaccharidosis (types I and II) and Pompe disease were added to the mass screening program in March 2025.

Trends in major development pipelines

Market size for the HGF gene therapy product estimated at over ¥200.0bn in the US alone

The Company's main development pipelines include the HGF gene therapy product, NF- κ B decoy oligonucleotide DNA, and a Tie2 receptor agonist being co-developed with its partner Vasomune.

Status of major development pipelines

Project	Region	Licensee/Partner	Indication	Development Stage
HGF gene therapy product	Japan	-	Chronic arterial occlusive disease	In June 2024, the Company withdrew its application for full approval seeking removal of conditional and time-limited marketing authorization conditions. Discussions with the PMDA to resume after marketing approval is obtained in the US
	US	-	Chronic limb-threatening ischemia	The late Phase 2 clinical trial (75 cases) was completed in March 2023, Breakthrough Therapy Designation was granted by the FDA in September 2024, and the principal investigator announced topline data at an academic conference in November 2024. Preparations underway for BLA submission
	Israel	Kamada Ltd.	Chronic arterial occlusive disease	Following the withdrawal of the application for full approval in Japan, Kamada also withdrew its marketing authorization application
	Turkey	ER-KIM Pharmaceuticals Co., Ltd.	Chronic arterial occlusive disease	Following the withdrawal of the application for full approval in Japan, ER-KIM also temporarily suspended preparations for its application
NF- κ B decoy oligonucleotide DNA	US	-	Chronic discogenic low back pain	A late Phase 1 clinical trial in the US was completed (25 cases, 2018–2021)
	Japan	Shionogi & Co., Ltd.	Chronic discogenic low back pain	In Japan, the Company entered into an agreement with Shionogi & Co., Ltd. for collaboration on a Phase 2 clinical trial. Commenced in October 2023 (planned enrollment: 92 cases)
DNA vaccine	Australia	-	Hypertension	A Phase 1/2a clinical trial in Australia was completed (24 cases, 2018–2021). Measures to address technical issues are under consideration
Tie2 receptor agonist AV-001	US	Vasomune Therapeutics, Inc.	ARDS	A joint development agreement was signed with Vasomune in July 2018, and an early Phase 2 clinical trial was initiated in North America in January 2022 (planned enrollment: 60 cases). Fast Track designation was granted by the FDA in May 2024

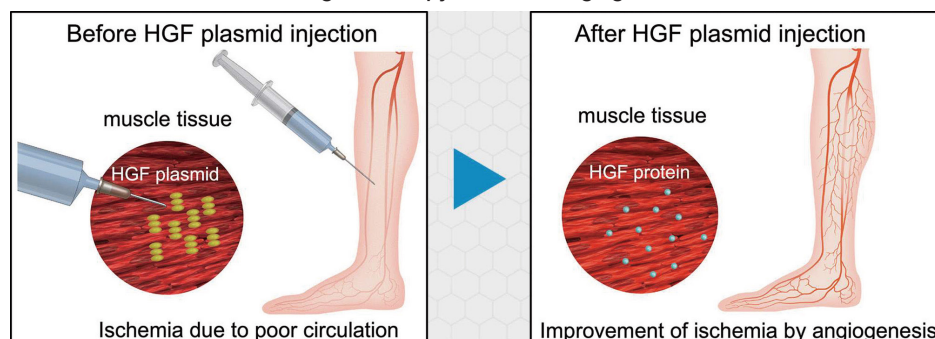
Source: Prepared by FISCO from the Company's financial results and results briefing materials

1. HGF gene therapy products

The HGF gene therapy product has been developed as a treatment for patients with advanced chronic arterial occlusive disease by leveraging its angiogenic effects. Chronic arterial occlusive disease is a serious condition in which blood vessels become blocked, leading to impaired blood flow, resulting in tissue ulcers and gangrene, and in some cases ultimately requiring limb amputation. Current treatment options include catheter-based interventions and vascular bypass surgery; however, many cases are ineligible for surgery, and the development of new therapeutic approaches is therefore required. The HGF gene therapy product is administered via multiple injections around the site of the vascular occlusion to induce new blood vessel formation and restore blood flow, thereby promoting the improvement of ulcers.

Trends in major development pipelines

HGF gene therapy-mediated angiogenesis

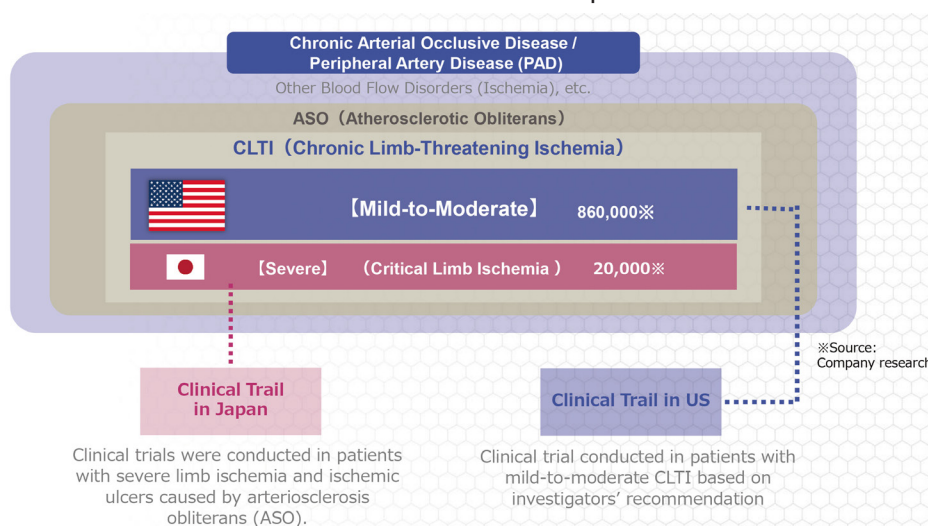


Source: The Company's results briefing materials

In the US, based on the Global Vascular Guideline* for chronic limb-threatening ischemia revised in June 2019 and proposals from the principal investigators, the Company conducted a late Phase 2 clinical trial targeting patients in clinical stage one or two with a lower risk of lower-limb amputation. The trial was designed based on the hypothesis proposed by the principal investigator that, as with cancer, early initiation of treatment is important for patients with critical limb ischemia.

* The Global Vascular Guideline (hereafter, "GVG"): recommends improving patients' QOL by providing appropriate treatment management from the early stages of Chronic Limb-Threatening Ischemia (hereafter, "CLTI"). The guideline classifies clinical stages into four levels (clinical stages one to four) and outlines treatment strategies for each stage. The late Phase 2 clinical trial conducted in the US targeted clinical stages one and two, which carry a lower risk of lower-limb amputation. For patients at these stages, the guideline recommends that treatment of ulcers should be considered first.

Disease indications in clinical trials in Japan and the US



Source: The Company's results briefing materials

Trends in major development pipelines

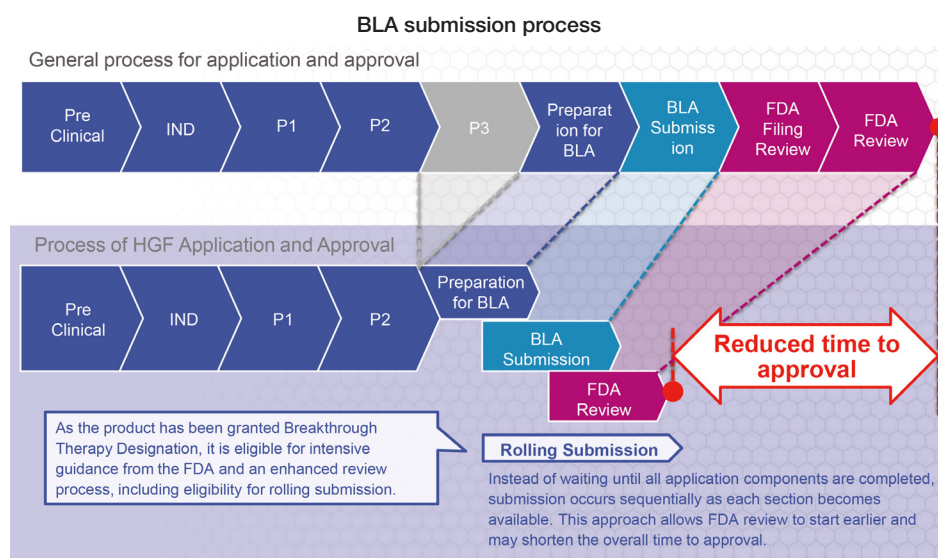
The primary endpoints were “time to healing” and “the proportion of completely healed ulcers at six months after administration,” and a placebo-controlled, randomized, double-blind study was conducted in which either the HGF gene therapy product or placebo was administered four times at four-week intervals. Subjects were divided into 3 groups: 4mg per dose, 8mg per dose, and placebo, and data was collected over a 12-month observation period (a total of 75 subjects were enrolled, including allowances for dropouts). A paper by the principal investigator summarizing the trial results was published in November 2025 in “Circulation: Cardiovascular Interventions,”* a journal issued by the American Heart Association (AHA). Specifically, the results confirmed that the time to healing was 84 days in the treatment group versus 280 days in the placebo group ($p=0.007$), the proportion of completely healed ulcers at 6 months was 63.3% in the treatment group compared with 38.5% in the placebo group ($p=0.053$), and the proportion of healed ulcers at 12 months was 77.6% in the treatment group versus 46.2% in the placebo group ($p=0.010$). Based on the above results, the HGF gene therapy product was concluded to significantly shorten the time to complete ulcer healing in patients with moderate CLTI and may represent a promising non-surgical treatment option.

* This is a peer-reviewed journal focused on cardiovascular intervention, a field that researches and practices minimally invasive treatments for cardiac and vascular diseases using catheter-based techniques. It is frequently cited as a reference in other academic publications and is recognized as a leading journal with significant influence.

Based on these trial results, the product received Breakthrough Therapy Designation* from the FDA, and after further discussions with the FDA, the Company decided in August 2025 to complete the clinical trial and seek commercialization through a BLA submission. In connection with this decision, the Company entered into a contract development and manufacturing agreement with Boehringer Ingelheim Biopharmaceuticals GmbH (hereafter, “Boehringer”), the contract manufacturer of the HGF gene therapy product, to establish a drug substance manufacturing framework for approval and commercialization. Until now, the drug substance had been manufactured and supplied using small-scale facilities; however, commercialization would require the construction of large-scale culture facilities, meaning that a certain amount of time and investment will be needed before the manufacturing framework is in place. Boehringer is establishing a Chemistry, Manufacturing and Controls (CMC) framework covering formulation, manufacturing processes, and quality control in preparation for the BLA submission. Under this designation, the BLA submission documents (nonclinical, clinical, and CMC) can be submitted sequentially as each section becomes ready, enabling the review to start earlier. The Company plans to submit the initial application documents to the FDA in 3Q FY12/26, with the CMC information related to drug substance manufacturing by Boehringer to be submitted as the final part of the application in 1Q FY12/27. The approval decision will be known six months after submission of the final application documents, and if the process proceeds smoothly, the Company could potentially commence sales within 2027.

* The Breakthrough Therapy Designation is a system introduced by the FDA to expedite the development and review of new therapies for serious or life-threatening diseases. It is granted to drug candidates that, based on clinical trial data, demonstrate the potential to provide substantial improvement over existing treatments.

Trends in major development pipelines



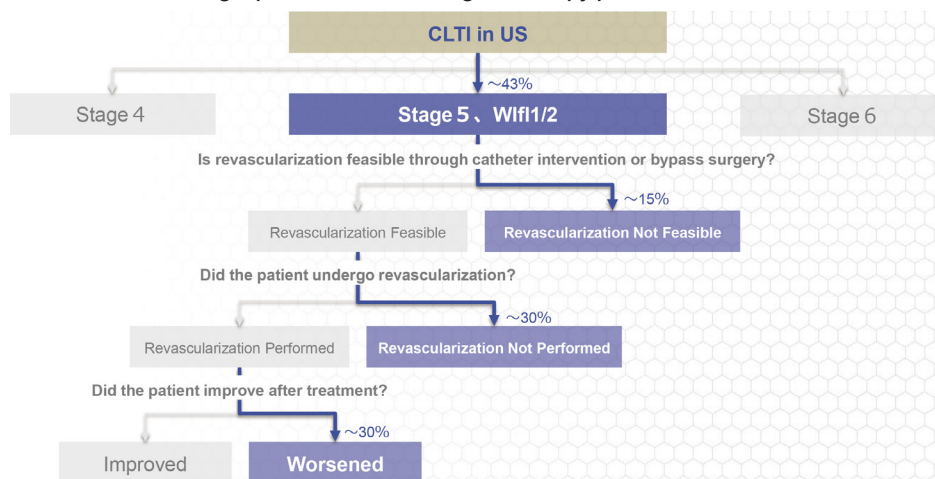
Source: The Company's results briefing materials

A key factor in launching the product will be negotiations on sales licensing with major pharmaceutical companies that have established sales networks. The Company first concluded non-disclosure agreements (NDAs) and began by providing data at the working level. It plans to hold discussions between top management from September 2026 onward, sign an LOI around December 2026, and conclude a definitive agreement in 1Q FY12/27. The Company is focusing its consideration on global companies with strengths in diabetes therapeutics and the cardiovascular area, where indications overlap and synergies can be expected, and aims to conclude an agreement with the support of US specialist physicians. If an agreement is concluded, the Company will receive upfront payments, milestone payments, royalty income based on post-launch sales, and other income. Taking its financial position into account, the Company intends to pursue negotiations in a manner that allows it to secure funds as early as possible. With regard to the terms of the agreement, the Company may also pursue partner negotiations with a view to global expansion, including the European market. In Japan, the Company plans to hold discussions with the Pharmaceuticals and Medical Devices Agency (PMDA) after obtaining marketing approval in the US.

Upfront payments and milestone payments are often calculated by applying a certain percentage to peak sales. FISCO estimates that the market size for the HGF gene therapy product exceeds ¥200.0bn in the US alone, and the upfront payment is also expected to be substantial. According to a research firm, the number of eligible patients in the US is estimated at a maximum of approximately 500,000 per year. Of the approximately 2 million patients with CLTI, patients with mild-to-moderate disease (Rutherford stage 5, Wifl 1/2) eligible for the HGF gene therapy product account for up to 43%, or approximately 860,000 patients. Of these, the target population comprises patients who are unable to undergo revascularization procedures such as bypass surgery for any reason, patients who have refused revascularization, and patients whose condition has worsened after revascularization, totaling up to approximately 500,000. In contrast, the Company estimates the actual number of eligible patients at 200,000–300,000, and assumes that 20% of these patients (40,000–60,000) will use the HGF gene therapy product. The drug price is expected to be benchmarked against the US\$75,000 cost of a revascularization procedure, but taking the burden on patients into account, a price of around US\$40,000–45,000 appears appropriate. Multiplying this by the assumed number of patients yields US\$1.6–2.7bn, which could translate into more than ¥200.0bn in yen terms.

Trends in major development pipelines

Target patients for the HGF gene therapy product in the US



Source: The Company's results briefing materials

The results of the Phase 2 clinical trial for chronic discogenic low back pain are expected to be announced around the end of 2027

2. NF- κ B decoy oligonucleotide DNA

NF- κ B decoy oligonucleotide DNA is a type of nucleic acid medicine that regulates gene function using synthetic nucleic acids. It is a specific inhibitor of a transcription factor protein (NF- κ B) that induces immune and inflammatory responses in the body. NF- κ B binds to specific DNA sequences in genes and activates their expression, leading to the production of proteins that cause inflammation, including pain. By introducing NF- κ B decoy oligonucleotide DNA into cells, the binding between NF- κ B and gene regions responsible for producing inflammatory proteins is inhibited, thereby suppressing the production of such proteins.

The Phase 2 clinical trial in Japan of NF- κ B decoy oligonucleotide DNA was initiated in October 2023 for chronic discogenic low back pain*, following the conclusion of a collaboration agreement with Shionogi & Co., Ltd. in March 2023, under which the Company partially bears the cost of the clinical trial. The trial plans to enroll 92 patients in total. An initial safety test at the maximum dose of 20mg was performed in the first 2 cases, followed by an ongoing comparative trial with three groups—10mg, 20mg, and placebo—each consisting of 30 patients receiving a single dose. The observation period is 12 months, and efficacy is evaluated based on changes in the Numerical Rating Scale (hereafter, "NRS"), an indicator of pain. Enrollment is expected to be completed within 2026. Although this is taking longer than initially anticipated, it is because the Company is carefully selecting target patients in order to appropriately evaluate the efficacy of the product. If the trial proceeds smoothly, the clinical trial results are expected to be announced around the end of 2027. In the event of positive results, the Company intends to pursue out-licensing.

* Chronic discogenic low back pain refers to low back pain caused by intervertebral disc disorders that persists for more than three months. It is estimated to account for approximately 40% of chronic low back pain cases. Eligible clinical trial participants are patients aged 18–75 whose NRS score for low back pain (a self-reported pain scale) is higher than their NRS score for buttock pain or lower-limb pain. The study targets patients with an inadequate response to conservative treatment for low back pain whose NRS scores at screening and on both the administration day and the preceding day range from four to nine, indicating moderate to severe pain. In addition, patients experiencing pain in multiple locations are excluded from the study.

Trends in major development pipelines

The results of a late Phase 1 clinical trial conducted in the US from 2018 ahead of the clinical trial in Japan (a placebo-controlled, randomized, double-blind comparative study involving 25 patients with a 50-week observation period) were published in May 2025 in The Spine Journal*, a specialist academic journal focused on spinal disorders. The trial confirmed no safety or tolerability concerns. Regarding efficacy, a dose-dependent analgesic effect was demonstrated across the three dose groups (0.3mg, 3mg, and 10mg), and the highest-dose 10mg group showed marked improvement in low back pain soon after administration, with pain scores decreasing by an average of 77% from baseline after 1 year (compared with an average of 40% in the placebo group). Furthermore, no cases in this dose group required supplemental analgesic treatment throughout the trial period, indicating the persistence of the analgesic effect. More specifically, pain almost completely disappeared in half of the patients in this dose group, and while intervertebral disc height decreased in the placebo group, an increase was observed in the 10mg dose group, suggesting recovery of the intervertebral discs. As a dose-dependent analgesic effect was confirmed in the US trial and the clinical trial in Japan also includes a 20mg dose group exceeding the maximum 10mg dose used in the US trial, FISCO believes there is a high likelihood of obtaining favorable results.

* The Spine Journal is a leading international academic journal in the field of spinal disorders published by the North American Spine Society. It features research papers, reviews, and case reports related to spinal surgery and provides high-quality peer-reviewed articles from around the world on a biweekly basis.

In Japan, symptomatic treatments such as oral and topical medications and physical therapy are commonly used for patients with chronic discogenic low back pain. However, NF- κ B decoy oligonucleotide DNA is expected to provide sustained efficacy for at least one year with a single administration, thereby contributing to improved patient QOL. If successfully developed, it could become the world's first nucleic acid therapeutic for chronic discogenic low back pain, and attention is focused on the clinical trial results expected to be announced around the end of 2027.

Clinical trial results for AV-001, a treatment for ARDS, are expected to be announced within 2026

3. ARDS therapeutic drug (Tie2 receptor agonist compound)

AV-001 (a Tie2 receptor agonist)*, an ARDS therapeutic co-developed with Vasomune, has been under joint global development since 2018 as a treatment for diseases caused by vascular dysfunction, including acute respiratory failure. Believing that the drug could also be effective for patients with moderate to severe COVID-19 pneumonia, the Company began an early Phase 2 clinical trial in the US in January 2022. However, partly because the pandemic has subsided, the target indication has been expanded to ARDS, including viral and bacterial pneumonia such as influenza (approved by the FDA), and the clinical trial is currently proceeding under the expanded scope.

* In 2018, the Company entered into a global co-development agreement with Vasomune for AV-001, targeting diseases caused by vascular dysfunction, including acute respiratory failure. Under the agreement, development costs and future profits are shared equally, and the Company is required to pay Vasomune upfront payments and milestone payments based on development progress. There are approximately 260,000 ARDS patients in the US alone.

As of December 2025, enrollment of the initially planned 60 cases had been completed; however, additional enrollment is being conducted to compensate for dropout cases, and all enrollment is expected to be completed in 3Q FY12/26. Topline data is expected to be announced as early as within 2026, and if favorable results are obtained, the Company intends to pursue out-licensing. In May 2024, AV-001 was granted Fast Track designation* by the FDA, allowing clinical trial consultations and review processes to proceed more efficiently.

* The Fast Track designation system was established to promote the development and expedite the review of new therapies for serious diseases and drugs that may address unmet medical needs.

Trends in major development pipelines

Furthermore, an investigator-initiated clinical trial of AV-001 aimed at preventing acute ischemic brain injury in hemodialysis patients has newly commenced, with dosing of the first subject initiated in March 2026. The trial is being conducted with funding support from the Heart and Stroke Foundation of Canada and is designed to evaluate whether AV-001 can reduce cytotoxic cerebral edema caused by hemodialysis, suppress cognitive decline, and preserve white matter function in the brain. The planned enrollment is 60 cases, with completion of enrollment expected within 2027. Up to 90% of patients with end-stage renal disease undergo hemodialysis, and cytotoxic cerebral edema develops in roughly 70% of patients aged 55 years or older in particular, leading to moderate to severe cognitive dysfunction and posing a significant issue in clinical practice. By targeting the Tie2/Angiopoietin-1 signaling pathway, AV-001 stabilizes vasculature and inhibits vascular leakage and inflammation, which may reduce cytotoxic cerebral edema and offer a novel treatment option for preserving brain function in patients undergoing hemodialysis.

In connection with the initiation of this investigator-initiated clinical trial, the Company entered into an agreement with Vasomune in November 2025 to expand the scope of the joint development agreement for AV-001 to cover all indications. Under the agreement, the Company will pay US\$3mn between 2026 and 2027. However, if the product is out-licensed or successfully commercialized, the Company will also be entitled to receive a portion of the resulting proceeds.

The Company is also considering development for the resuscitation treatment of patients with severe burns, such as those injured in combat. Administration of AV-001 is intended to activate the Tie2 receptor, a transmembrane protein highly expressed on the surface of vascular endothelial cells, thereby suppressing vascular leakage and maintaining vascular endothelial barrier function to aid resuscitation treatment. The Company intends to advance development using a grant from the US Department of Defense. It is currently revising the protocol and will initiate a Phase 2 clinical trial once preparations are complete.

Development status of EmendoBio

The OMNI platform's strength lies in its strong safety profile among genome-editing technologies

1. Features of genome editing technology and the OMNI platform

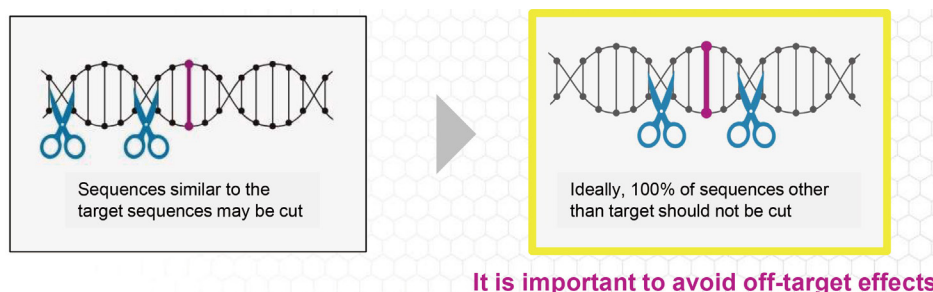
Genome editing refers to a technology that modifies target genes by using DNA-cleaving enzymes (nucleases) that cut only specific base sequences (target sequences). In 2012, the emergence of an innovative technology called CRISPR/Cas9, which enables targeted DNA sequences to be cut easily through a mechanism different from conventional methods, accelerated efforts within the pharmaceutical industry to develop new drugs using genome-editing technology, and in 2023 the first therapies using this technology were approved in the UK and the US*.

* A therapy for sickle cell disease, jointly developed by US-based Vertex Pharmaceuticals Inc. <VRTX> and Switzerland-based CRISPR Therapeutics <CRSP>, was approved. The treatment works by applying genome-editing technology to genetically alter hematopoietic stem cells harvested from the patient and subsequently administering them back into the body through injection. Sickle cell disease is a disorder in which hemoglobin, the protein in red blood cells responsible for transporting oxygen, becomes deformed due to a genetic abnormality, causing red blood cells to assume a sickle shape and become prone to destruction, resulting in anemia. In severe cases, it can lead to renal failure or heart failure.

Development status of EmendoBio

The first approval of a therapeutic developed using CRISPR/Cas9 technology has provided some confirmation of its safety. However, concerns remain regarding off-target effects, in which unintended regions of the genome may be cleaved non-specifically. In contrast, the OMNI platform independently developed by EmendoBio is a system for discovering and optimizing nucleases that perform highly efficient genome editing while minimizing off-target effects, and is a technology for creating novel nucleases unlike any that existed before. Patent applications have been filed for more than 250 internally developed nucleases. In the development of pharmaceuticals using genome-editing technology, both efficacy and safety are critical, and therefore FISCO believes that the OMNI platform is expected to provide a competitive advantage. According to research by EmendoBio, the market size for therapies targeting genetic disorders is estimated at approximately ¥2tn in total, of which approximately ¥1.1tn could fall within the addressable area of the OMNI platform, indicating significant growth potential.

Aims to avoid off-target effects



It is important to avoid off-target effects

Source: The Company's results briefing materials

Clinical trial initiation is anticipated in 2026 for development pipelines incorporating OMNI technology at partner companies

2. Business strategy

EmendoBio implemented a business restructuring in 2024 due in part to the prolonged conflict in Israel, and currently operates with a team totaling approximately 20 genome-editing researchers and IT engineers. Given its financial condition, EmendoBio is concentrating its business strategy on licensing activities for over 250 OMNI nucleases and the OMNI platform developed to date.

Regarding licensing agreements, in March 2024, EmendoBio signed a non-exclusive license agreement with Anocca AB*1 for the rights to use the OMNI-A4 nuclease. Anocca is a Sweden-based company that leads the industry in the development of TCR-T cell therapy*2, a form of cancer immunotherapy. Anocca is advancing development using the OMNI-A4 nuclease to target KRAS protein mutations in refractory solid tumors. In September 2025, the parties agreed to expand the scope of the licensing agreement. Anocca highly values the OMNI technology and is expected to actively utilize OMNI nucleases in its future development pipeline. Clinical trials for a development pipeline using OMNI-A4 are expected to commence within 2026, and the Company anticipates receiving milestone payments from Anocca. Under the original agreement, the total milestone payments, including the upfront payment, were up to US\$100mn, but this amount may increase following the expansion of the licensing scope.

*1 Anocca is a biotech venture established in 2014 with more than 100 employees, primarily consisting of scientists, engineers, and software developers. Anocca possesses technology for selecting and culturing T cells that express T-cell receptors recognizing specific antigens, and has a library of TCR-T cell therapies targeting multiple cancer antigens, with more than 40 product candidates in its pipeline.

*2 TCR-T cell therapy refers to a gene-modified T-cell therapy in which lymphocytes are harvested from a patient, engineered by introducing a cancer antigen-specific T-cell receptor into the T cells, and subsequently reinfused into the same patient. Development is ongoing for use in refractory solid tumors.

Regarding contract negotiations with other companies, a Swiss pharmaceutical company is currently conducting a technical evaluation of the OMNI platform, and if favorable results are obtained, this could lead to a licensing agreement as early as 2026. In January 2025, the Company entered into a joint research agreement with Stanford University in the US to develop novel cancer therapies using genome-editing technology. The research aims to develop a new treatment for hereditary refractory breast cancer by integrating Stanford University's intracellular drug delivery technology with OMNI nucleases from EmendoBio to enable cancer cell-specific genome editing, thereby reducing therapy resistance and selectively inducing cancer cell death. The research is expected to run for approximately 2 years with a budget of around US\$1.30mn. The research results are scheduled to be published in a paper upon completion of the joint research. Going forward, the development of OMNI nucleases will require greater expertise in computer science, and Stanford University has many highly capable engineers in this area. The Company is also considering hiring such talent and potentially relocating its research and development base to the US in the future.

■ Status of ACRL initiatives

Aims to expand the testing business by raising funds through alliances, organizational restructuring, and other measures

The expanded newborn screening tests for rare genetic diseases, which began in July 2021, have seen steady growth in test volume, with approximately 90,000 tests contracted in FY12/25. This was driven by testing services commissioned by CReARID, as well as the commencement of testing contracts with local governments (or related organizations) from August 2024 onward. In addition, following the termination of contract services by CReARID at the end of March 2025, the Company has been receiving testing orders directly from clinics, resulting in an increase in the unit price per test. However, as processing capacity has reached its upper limit in terms of testing equipment as well as human and financial resources, growth is expected to be limited to a slight increase in FY12/26.

As part of its efforts to expand its testing business, the Company began providing genetic testing (diagnostic testing) for rare genetic diseases in May 2024. Furthermore, in September 2025, the Company began providing biomarker testing for mucopolysaccharidosis, including secondary screening, follow-up observation, and monitoring of treatment efficacy, thereby establishing a system capable of offering one-stop testing services for rare genetic diseases. Traditionally, no single laboratory has performed all of these tests, requiring medical institutions to outsource to multiple laboratories, which has been time-consuming. The Company's ability to provide one-stop contract testing services is therefore highly significant. Although confirmatory testing and biomarker testing volumes are much lower and therefore have only a limited direct impact on earnings, conducting a greater number of tests for rare genetic diseases has the advantage of increasing opportunities to identify new therapeutic candidates.

While the Company continues to work on expanding its customer base and the range of diseases covered with the aim of growing the testing business, it currently positions the launch of the HGF gene therapy product as its top management priority and plans to concentrate funding on this priority, leaving it with no choice but to continue postponing capacity expansion investments and other initiatives. To break out of this situation, the Company has announced a policy of raising funds through alliances with other companies, organizational restructuring, and other measures in order to expand the business, and is currently in discussions with candidate companies.

Results trends

Operating loss widened in 1H FY12/26 due to higher R&D expenses

1. Overview of 1H FY12/26 results

For 1H FY12/26, business revenue was ¥502mn (up ¥87mn YoY), operating loss was ¥3,236mn (widened by ¥835mn), ordinary loss was ¥2,560mn (down ¥1,338mn), and loss attributable to owners of parent was ¥2,714mn (down ¥1,251mn).

Consolidated results for 1H FY12/26

	1H FY12/25	1H FY12/26	YoY		Key factors
			Change	% change	
Business revenue	414	502	87	21.2%	Zokinvy (+89), ACRL fee income (-1)
Business expenses	2,815	3,738	923	32.8%	
Cost of sales	254	265	10	4.0%	Zokinvy (+26), ACRL (-16)
R&D expenses	1,555	2,668	1,112	71.5%	Outsourcing expenses (+643), payment fees (+374)
SG&A expenses	1,004	805	-199	-19.8%	Payment fees (-198)
Operating profit/loss	-2,400	-3,236	-835	-	
Ordinary profit/loss	-3,898	-2,560	1,338	-	Foreign exchange gains/losses (+2,173)
Profit/loss attributable to owners of parent	-3,966	-2,714	1,251	-	

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

Within business revenue, product sales increased by ¥89mn YoY to ¥232mn due to an increase in the number of patients treated with Zokinvy, a treatment for progeria. On the other hand, fee income from contract testing services was limited to ¥270mn, down ¥1mn, as growth in the number of testing orders stalled.

Business expenses increased by ¥923mn YoY to ¥3,738mn. Although SG&A expenses decreased by ¥199mn, mainly due to lower payment fees resulting from reduced compensation paid to lawyers and other professionals and consultants, R&D expenses increased by ¥1,112mn. Outsourcing expenses increased by ¥643mn due to higher costs related to manufacturing testing of the HGF gene therapy product, and payment fees increased by ¥374mn due to higher contract service fees related to the FDA submission and other factors.

While the increase in R&D expenses related to the HGF gene therapy product contributed to the widening of the operating loss, a non-operating foreign exchange gain of ¥682mn was recorded (compared with a foreign exchange loss of ¥1,491mn in 1H FY12/25), mainly as a result of the revaluation* at the end of 1H FY12/26 of the US dollar-denominated loan of approximately US\$100mn extended to EmendoBio, which contributed to the narrowing of the ordinary loss.

* Valued at the exchange rate of ¥162 per US dollar at the end of June 2026, compared with ¥156 per US dollar at the end of December 2025.

Operating loss for FY12/26 may be smaller than the initial forecast

2. FY12/26 forecasts

For FY12/26, the Company has maintained its initial forecasts of business revenue of ¥1,330mn (up ¥456mn YoY), operating loss of ¥10,230mn (up ¥5,085mn), ordinary loss of ¥10,240mn (up ¥4,952mn), and loss attributable to owners of parent of ¥10,250mn (up ¥5,127mn).

Consolidated forecast for FY12/26

	(¥mn)		
	FY12/25 results	FY12/26 forecasts	YoY change
Business revenue	874	1,330	456
Operating profit/loss	-5,145	-10,230	-5,085
Ordinary profit/loss	-5,288	-10,240	-4,952
Profit/loss attributable to owners of parent	-5,123	-10,250	-5,127

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

Business revenue is expected to increase mainly due to growth in Zokinvy sales as the number of treated patients increases, as well as the recognition of milestone payments associated with the entry of Anocca's development pipeline into clinical trials. Fee income from expanded newborn screening tests is expected to increase only slightly due to capacity constraints.

On the cost side, SG&A expenses are expected to remain at a level comparable to the previous fiscal year. R&D expenses are projected to increase by approximately ¥5.5bn, mainly due to active pharmaceutical ingredient manufacturing costs for the HGF gene therapy product and regulatory submission preparation costs in the US, which will contribute to a widening of losses. However, given its tight financial situation, the Company has adopted a policy of minimizing expenses other than those related to the HGF gene therapy product, and the operating loss may therefore be smaller than forecast. In addition, as no upfront licensing payment for the HGF gene therapy product has been factored into the forecast, a meaningful upward revision to the forecast could be expected if a licensing agreement is concluded.

Policy of funding business activities for the time being through equity financing and other means

3. Financial position and financing plan

(1) Financial position

As of the end of 1H FY12/26, total assets decreased by ¥1,745mn from the end of the previous fiscal year to ¥3,660mn. Within current assets, cash and deposits decreased by ¥1,600mn to ¥281mn due to payments of business expenses, and consumption taxes receivable decreased by ¥104mn. Within non-current assets, property, plant and equipment decreased by ¥17mn, while investments and other assets increased by ¥39mn.

Results trends

Total liabilities increased by ¥1,124mn from the end of the previous fiscal year to ¥3,454mn. While income taxes payable decreased by ¥283mn, accounts payable increased by ¥413mn due to higher costs related to manufacturing testing of the HGF gene therapy product and other factors, and other accounts payable increased by ¥315mn due to higher contract service fees related to the submission to the US FDA. In addition, interest-bearing debt amounted to ¥621mn due to the issuance of bonds. Total net assets decreased by ¥2,870mn from the end of the previous fiscal year to ¥205mn. While share capital and capital surplus each increased by ¥270mn due to the exercise of stock acquisition rights, retained earnings decreased by ¥2,714mn due to the recording of loss attributable to owners of parent, and foreign currency translation adjustment decreased by ¥684mn.

Consolidated balance sheets

	(¥mn)			
	End of FY12/24	End of FY12/25	End of 1H FY12/26	Changes
Current assets	3,542	4,386	2,619	-1,766
(Cash and deposits)	1,707	1,882	281	-1,600
Non-current assets	1,125	1,019	1,040	21
Total assets	4,668	5,405	3,660	-1,745
Total liabilities	2,512	2,329	3,454	1,124
(Interest-bearing debt)	-	-	621	621
Total net assets	2,156	3,076	205	-2,870
<Management indicators>				
Equity ratio	44.0%	55.2%	3.5%	-51.7pp
Interest-bearing debt ratio	-	-	490.0%	-

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

(2) Financing plan

To secure funds for its business activities for the time being, on August 14, 2026, the Company announced the issuance of 2nd series unsecured convertible bonds with stock acquisition rights, the 47th series of stock acquisition rights (with exercise price adjustment provision), and the 48th series of stock acquisition rights, with GP Listed Company Investment Limited Partnership, a fund operated by Growth Partners, as the planned allottee, as well as the conclusion of a business alliance agreement with Growth Partners. Payment for all of these was completed on August 31, with ¥2.0bn raised through the convertible bonds (interest rate of 0.75%, conversion price fixed at ¥43.5), and from September onward the Company plans to raise funds as needed through the exercise of the stock acquisition rights. The 47th series of stock acquisition rights has an initial exercise price of ¥50 (minimum exercise price of ¥25), while the exercise price of the 48th series of stock acquisition rights is fixed at ¥43.5. The total number of potential shares is 182,976 thousand, representing a dilution ratio of 45.88%*.

* The denominator is the 398,835 thousand shares outstanding as of the end of June 2026.

If all of the stock acquisition rights are exercised at the initial exercise prices, the total amount paid in, including the ¥2.0bn convertible bonds, would be ¥8,500mn, with estimated net proceeds of ¥8,196mn after deducting issuance costs. Since the announcement of the plan, the share price has fallen to ¥39 (closing price on August 24, 2026), and there is a possibility that the fundraising may not proceed as expected. However, the Company's policy is to fund its business activities through additional fundraising even after the exercise of the 48th series of stock acquisition rights. FISCO believes that if the licensing negotiations for the HGF gene therapy product are successfully concluded, this would serve as a catalyst for a rise in the share price and enable the Company to emerge from its current difficult situation. Attention will focus on whether the conclusion of an LOI or similar agreement, as a preliminary step toward the definitive agreement, is announced within 2026.

Results trends

The business alliance with Growth Partners aims to strengthen the Company's business foundation and enhance corporate value by leveraging Growth Partners' network and expertise and obtaining its cooperation in areas such as business alliances, capital policy, and business development.

Breakdown of the use of funds raised

Use of funds	Amount (¥mn)	Expected timing of expenditure
Redemption of the 2nd series unsecured bonds (private placement bonds)	630	August 2026–September 2026
Development costs for the HGF gene therapy product in the US	3,500	August 2026–October 2031
Costs of relocating EmendoBio's R&D base	1,000	August 2026–October 2031
Working capital, including other R&D expenses	3,066	August 2026–October 2031
Total	8,196	

Note: The 2nd series unsecured bonds (private placement bonds) were issued in February 2026 and redeemed on September 1, 2026
Source: Prepared by FISCO from the Company's IR news

Future growth strategy

Aims to become a global leader in gene therapeutics

The Company has significantly advanced the development of its lead product, the HGF gene therapy product, in the US and decided to proceed with preparations for a BLA submission. In light of this milestone, it revised its corporate philosophy and redefined its mission, vision, and values.

Specifically, the Company defined its mission as focusing on the development of gene therapeutics leveraging the power of genes and research and development of treatments using next-generation technologies such as genome editing, with the aim of providing treatment opportunities to all patients suffering from rare and intractable diseases. As its vision, the Company aims to be a global leader in gene therapeutics that drives the world forward in gene medicine and genome editing, globally deploying multiple innovative therapies and contributing to improving the QOL of people worldwide. To realize this vision, the Company defined its values as maintaining a spirit of challenge without fear of change and valuing its network with all stakeholders, while continuously incorporating cutting-edge research findings and ideas, and pursuing the creation of new technologies and product value with the highest ethical standards and a strong sense of urgency.

The Company seeks to drive revenue growth and improve corporate value by reinforcing organizational capabilities and talent development for global expansion, as well as deepening and broadening its platform technologies, while pursuing initiatives such as maximizing the value of its HGF gene therapy product, continuously expanding its pipeline, accelerating global development focused on the US and Europe, and strengthening its approach to rare genetic diseases, including its diagnostics business. By conducting testing in the field of rare genetic diseases, the Company aims to identify new drug candidates in this disease area and create a virtuous cycle in which therapeutics are developed using EmendoBio's OMNI platform. FISCO believes that if the Company can establish such a cycle, it will move closer to achieving its vision of becoming a "global leader in gene therapeutics."

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