AnGes

FY2024 Financial Results Materials

— Leading Global in Gene Medicine —

February 2025

◆ **The performance forecasts and forward-looking statements in these materials are based on information currently available to the Company and include potential risks and uncertainties.**

◆ **These risks and uncertainties include changes in the economic environment surrounding the Company, progress with research and development, the approval of acquisitions by the regulatory authorities, and system changes and revisions to laws and regulations in countries around the world.**

◆ **Actual business performance and results may differ significantly from the described forecasts due to various factors.**

This document has been translated from the Japanese original for reference purposes only.
In the event of any discrepancy between this translation and the Japanese original, the original shall prevail.

Agenda

1 Summary of Financial Results for FY2024

2 Topics

①HGF Gene Therapy Product

②Zokinvy

③ACRL

3 Other developed products, Collaboration with partner companies

④EmendoBio Genome Editing

⑤Tie2 Receptor Agonist

01

Summary of Financial Results for FY2024

Highlights of Consolidated Results for FY2024

(Million Yen)

	Y2023	FY2024	Increase /decrease	FY2024 Earning Forecast	FY2024 Result	Increase /decrease
Revenues	152	**643**	490	600	**643**	43
Business Expenses	12,120	**9,753**	-2,367			
Operating Profit	-11,967	**-9,109**	2,858	-8,450	**-9,109**	-659
Non-operating income/expenses	6,316	**1,571**	-4,744			
Ordinary Profit	-5,651	**-7,537**	-1,886	-8,450	**-7,537**	912
Extraordinary income/losses	-1,820	**-20,105**	-18,285			
Profit	-7,437	**-28,128**	-20,691	-8,650	**-28,128**	-19,478

- The increase in revenue for the period was due to the start of sales of Zokinvy and a steady increase in the number of contracts for contract testing services.
- Ordinary income (loss) was negative year-on-year due to the absence of subsidy income from the previous fiscal year, and positive year-on-year due to the weaker yen.
- Significant net loss due to extraordinary loss resulting from goodwill impairment

Extraordinary Losses

2020/12

AnGes acquired EmendoBio with proprietary technology in genome editing technology, as a subsidiary

Approximately 22.7 billion yen of “goodwill” was recorded

2025/2

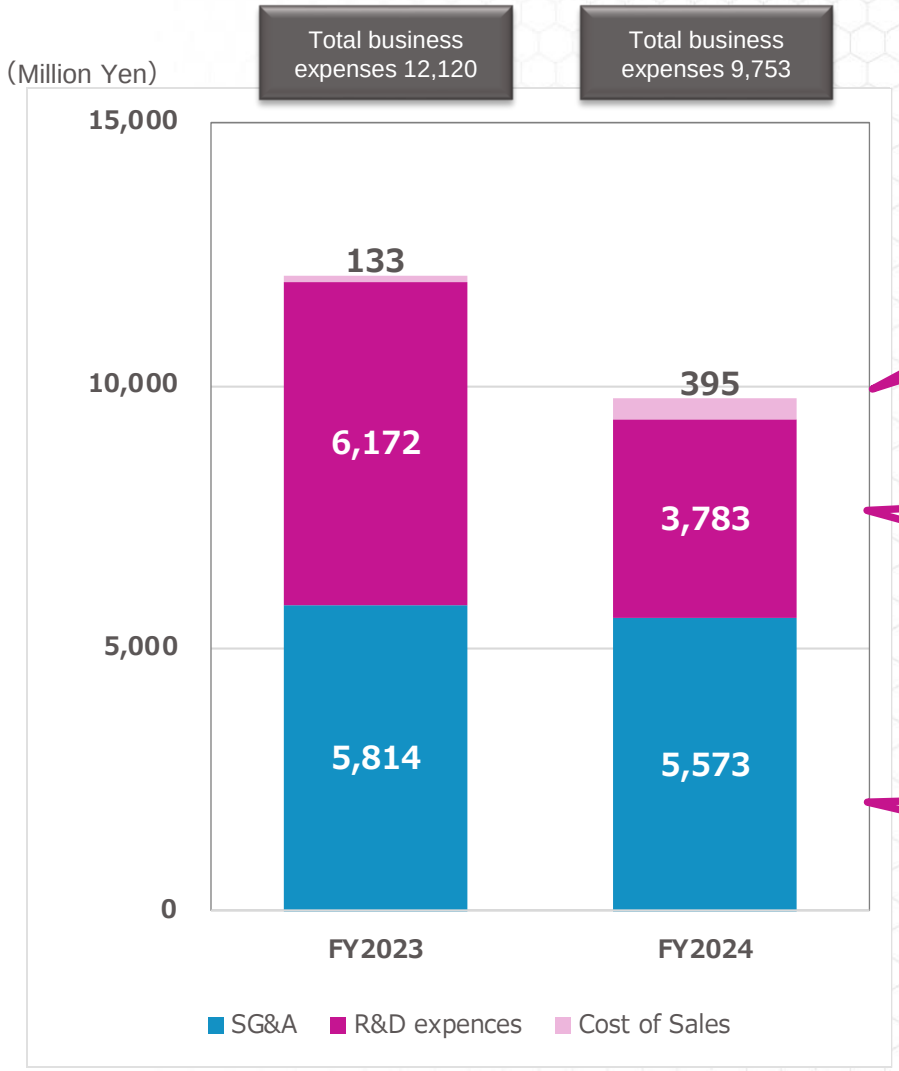
Impairment of non-current asset and goodwill in line with future activities from the launch of the new organization due to business restructuring, resulting in an extraordinary loss of 20.1 billion yen

Future

Strengthen licensing activities in the U.S.
Launched a joint research project with Stanford University

Accelerate performance improvement

Details of Business Expenses



Cost of Sales：395 (million Yen)

（YoY +262）
Increase in contracted testing service (YoY +113)
Cost of goods sold by Zokinvy (YoY +160)

R&D expenses： 3,783 (million Yen)

（YoY -2,389）
Mainly due to Emendo's restructuring
① Personnel expenses （YoY -1,152）
② Outsourcing expenses （YoY -724）
Fees paid for the introduction of Zokinvy (YoY -156)

SG&A： 5,573 (million Yen)

（YoY -240）
① Emendo-related rewards (YoY -244)
② Fees and commissions (YoY -137)
③ Amortization of goodwill（YoY +241）

Consolidated Balance Sheet Highlights

(Million Yen)

	Dec. 31, 2023	Dec. 31, 2024	Increase /Decrease
Current assets	5,921	**3,542**	-2,378
Cash and deposits	4,160	**1,707**	-2,452
Non-current assets	22,971	**1,125**	-21,845
Goodwill	21,746	**0**	-21,746
Total assets	28,892	**4,668**	-24,223
Liabilities	2,789	**2,512**	-277
Net assets	26,103	**2,156**	-23,946

Current assets

■Cash and Deposits 1,707 (-2,452)
4,425 in financing, but decreased due to project expenditures
■Goods 224(+224) By Zokinvy sales
■Raw materials and supplies (-264)
Appraisal loss

Non-current assets

■Goodwill 0 (-21,746) Impairment
■Property, Plant and Equipment 174 (-248)
Impairment of right-of-use assets

Liabilities

■Accounts payable (-118)
■Arrears (-167)
■Allowance for business restructuring (-391)
■Income taxes payable (+578)

Net assets

■Capital stock/capital surplus (+4,279)
■Retained earnings due to loss for the year (-28,128)
■Foreign currency translation adjustments (-130)

Earning Forecast for FY2025

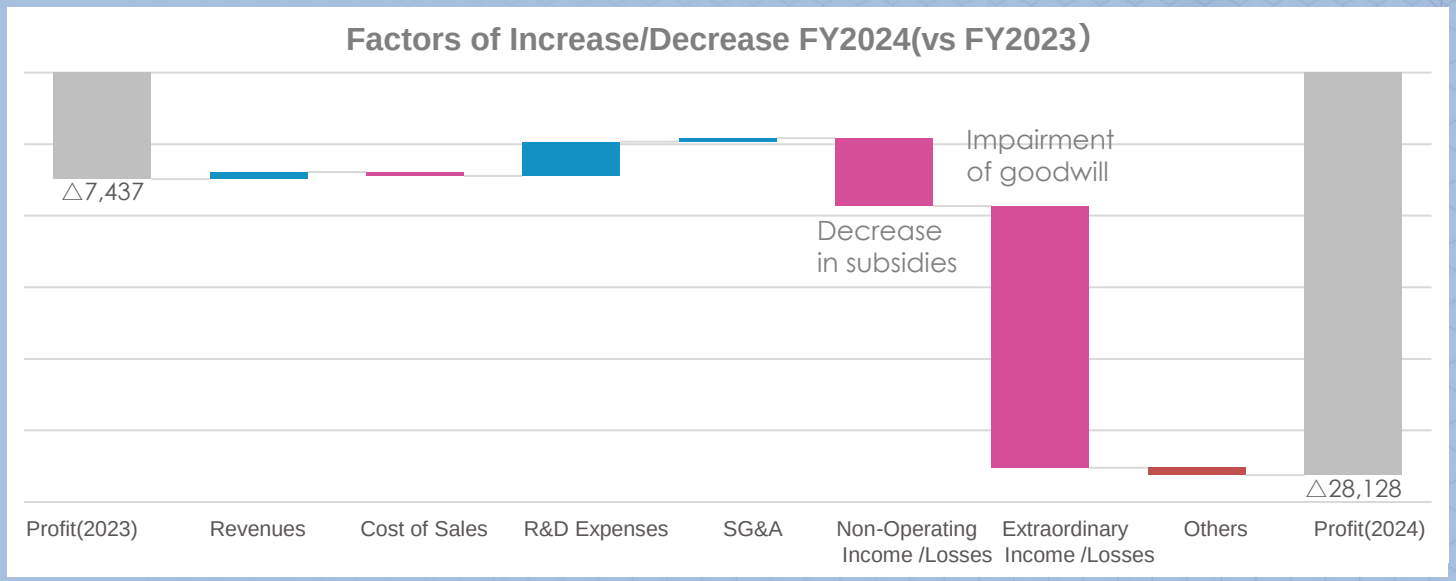
(Million Yen)

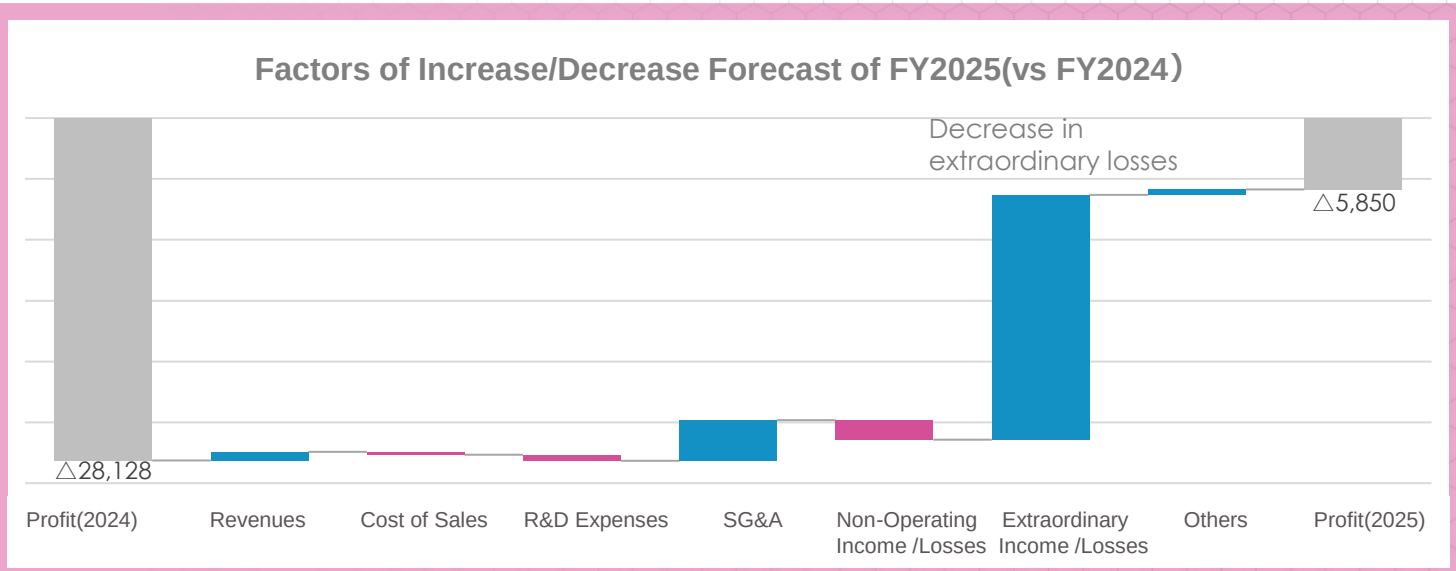
	Business Revenues	Operating Profit	Ordinary Profit	Profit
FY2025 full-year plan	**1,350**	**-5,800**	**-5,820**	**-5,850**
FY2024 full-year results	643	-9,109	-7,537	-28,128
Increase / decrease	707	3,309	1,717	22,278

Key Points of Full-Year Earnings Forecast FY2025

- Zokinvy sales planned throughout the year.
- Increase in the number of expanded newborn screening contracts at ACRL
- Reduction of expenses (including amortization of goodwill) associated with the restructuring of Emendo's business
- The extraordinary loss recorded in FY2024 will be eliminated.

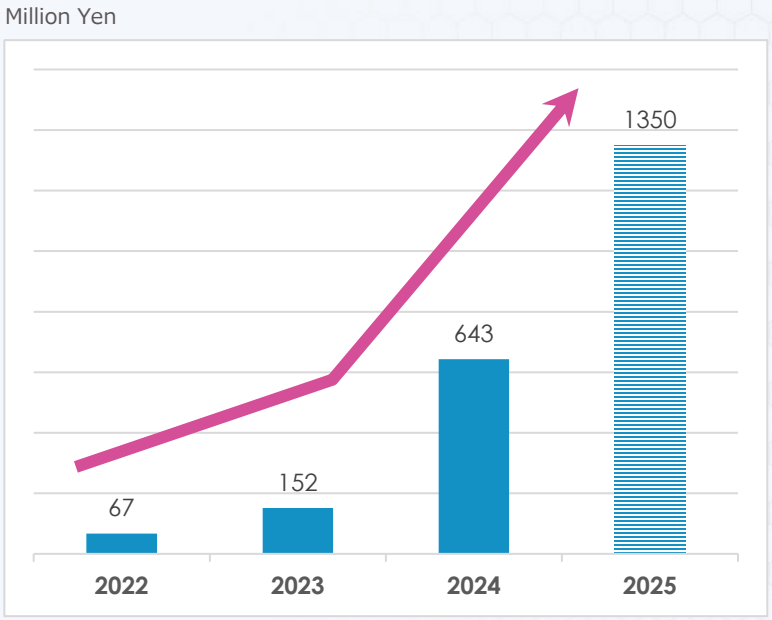
Factors of Increase/Decrease in Net Income(Loss)



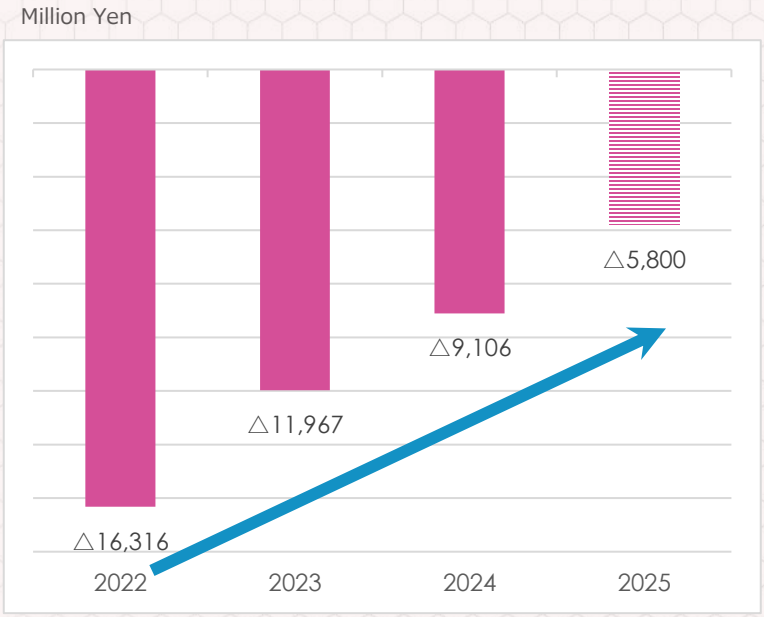


Financial Results

Revenues



Operating Income



Revenues increased

- Full-year contribution from sales of Zokinvy
- Increase in the number of expanded newborn screening contracts at ACRL

Operating income improved

- Reduction of expenses associated with Emendo's restructuring
- Profitability improvement due to the elimination of goodwill amortization

02

Topics

Status of Projects in the Clinical Development Stage

■ Approval Process

Project	Area	Licensee /partner	Dosage form	Indications	Basic research	Preclinical study	Clinical investigation (trial)			Approval/review	Approval
							Phase I	Phase II	Phase III		
HGF gene therapy product	Japan	–	Injectable	chronic arterial occlusive disease						P2b (Completed)	
	United States	–	Injectable	chronic arterial occlusive disease				P2b (Completed)			
	Israel	Kamada	Injectable	chronic arterial occlusive disease							
	Turkey	Er-Kim	Injectable	chronic arterial occlusive disease							
NF-κB decoy oligonucleotide DNA	US / Japan	–	Injectable	Chronic discogenic lumber back pain				P2b (On going)			
DNA vaccine	Australia	–	Injectable	Hypertension			Compl eted				
DNA vaccine	US	–	Intranasal formulation	COVID-19 / ADRS	Completed						
Tie-2 receptor agonists	United States	Vasomune	Injectable					P2a (on going)			
Zokinvy (lonafarnib)	Japan	Sentynl (Origin of in-licensing)	Capsule	Progeria (HGPS・PDPL)	In-licensed product					Approved now on sale	

FY2024 Topics

1. Progress of HGF gene therapy products
2. Zokinvy is now available in Japan
3. Testing services at ACRL

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HGF gene therapy product

Gene Therapy

A treatment that cures a disease through the action of a protein made from a specific gene placed in the patient's body

HGF

Promotes proliferation of hepatocytes

In 1984, a growth factor was discovered in Japan from the liver, the organ with the greatest regenerative capacity, and was named Hepatocyte Growth Factor (HGF).
HGF was found to play a major role in the formation and regeneration of various organs and tissues in the body, including blood vessels and nerves, as well as the liver.

HGF regenerates blood vessels

In 1995, Prof. Ryuichi Morishita and his research team at Osaka University discovered that HGF regenerates blood vessels, leading to the development of HGF gene therapy, a treatment for ischemic diseases caused by deteriorated blood flow.

History of HGF gene therapy product

<Japan>

November 2014
Conditional and time-limited approval systems come into effect

January 2018
Filed for manufacturing and marketing approval

March 2019
Received conditional and time-limited approval

September 2019
Launched by Mitsubishi Tanabe Pharma

May 2023
Filed for Full Manufacturing and Marketing Approval

June 2024
Withdrawal of application for approval
Priority will be given to the application for approval in the U.S., and future policies will be considered

<U.S.>

2014

2014～2016
Global Phase 3 Study

2019

June 2019
New Global Treatment Guidelines (GVG) published

February 2020
Enrollment begins for phase 2b clinical trials

2023

March 2023
Patient enrollment completed for phase 2b clinical trial

2024

June 2024
Positive results of phase 2b clinical trials confirmed

September 2024
Designated as Breakthrough Therapy by FDA

November 2024
Clinical Trial Results Presented at AHA Scientific Sessions

Main progress in 2024

September 2024

Designated as a **Breakthrough Therapy by the FDA** based on positive results of stage Phase 2b clinical trials in the U.S.

Expected to
shorten the review period and
improve the certainty of approval

November 2024

Clinical Trial Results Presented at **AHA Scientific Sessions**

Recognized for its clinical importance by a world-renowned and prestigious society in the field of cardiovascular medicine

Mortality after 5 years of Critical Limb Ischemia

Critical limb ischemia

According to Dr. Armstrong of the University of Southern California, the five-year mortality rate for patients with critical leg ischemia in the U.S. is 57%, a severe disease that is second only to the 80% mortality rate for lung cancer

Critical limb ischemia, like cancer treatment, should be started as early as possible

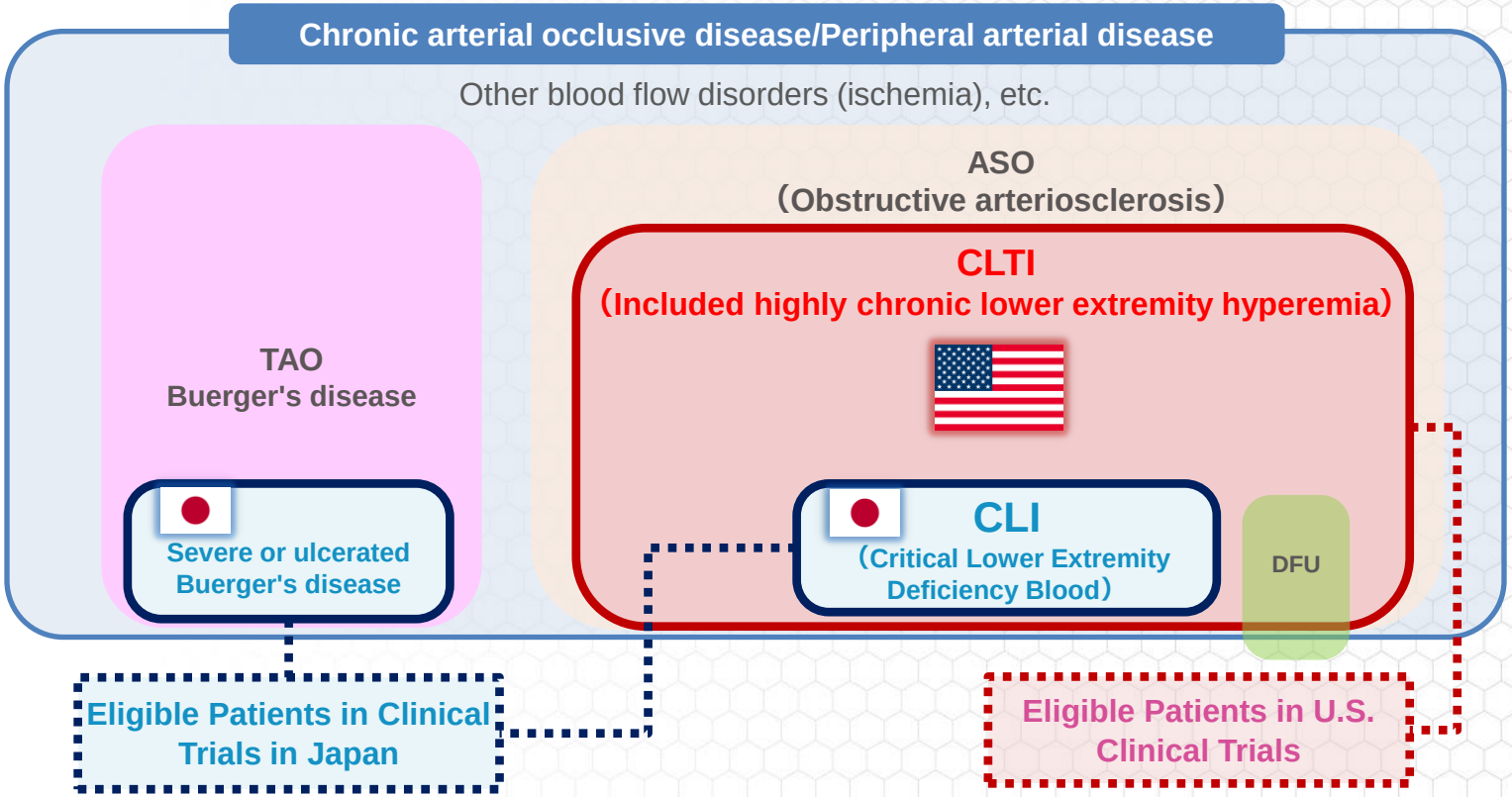
Phase 2b Clinical Trial in Patients with Chronic Arterial Occlusive Disease at Low Risk of Lower Limb Amputation

Achieve excellent clinical trial results

Papers to be published in prestigious medical journals

Scope of Diseases in Clinical Trials

Phase 2b clinical trial to expand target patients from CLI to CLTI based on 2019 guideline revision and clinical trial supervisor's suggestion



Patients with CLI with ulcers or TAO with ulcers in arteriosclerosis obliterans

Clinical trial for CLTI patients at low risk of lower limb amputation

FY2024 Topics

1 Progress of HGF gene therapy products

2 Zokinvy is now available in Japan

3 Testing services at ACRL

About Zokinvy

Start of sales on May 27,2024

2022 May — Exclusive distribution agreement signed with Eiger for Zokinvy in Japan

2022 May — Application for Orphan Drug Designation

2023 March — Received Orphan Drug Designation

2023 May — Submitted an application for manufacturing and marketing approval of “Zokinvy” in Japan

2024 January — Obtained manufacturing and marketing approval in Japan

2024 May — Start of Sales in Japan

Zokinvy

About Zokinvy

Zokinvy is a drug for the treatment of Hutchinson-Gilford Progeria Syndrome (HGPS) and processing-deficient progeroid laminopathy (PDPL)

Target diseases

“HGPS” and “processing failure PL”, known as Progeria Infantilis. Assumption is that the number of patients to be used will start with a few.
Progeria is a general term for a disease in which the signs of aging occur earlier than the actual age of the patient and are seen throughout the body. The average age of HGPS is reported to be 14.5 years.

Efficacy

Data show a 72% reduction in mortality and an increase in mean survival of about 4 years in patients with HGPS

Safety

Many HGPS patients have been on Zokinvy treatment for more than 10 years, and the most common side effects reported are vomiting, diarrhea, and nausea, most of which are mild or moderate

Zokinvy is an opportunity to eliminate drug loss/lag

The success of the introduction of “Zokinvy” was introduced in news programs and other media with the hope that it will serve as a catalyst for the elimination of drug loss and drug lag in Japan in the future.

A press briefing was held on June 18 to coincide with the launch of Zokinvy

Dr. Kenji Ihara, Professor of Oita University School of Medicine, and Dr. Muneaki Matsuo, Professor of Saga University School of Medicine, explained the characteristics of HGPS and PDPL, the target diseases of “Zokinvy”, and their treatment.

FY2024 Topics

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ACRL's Immediate Initiatives

AnGes Clinical Research Laboratory (ACRL) offers
“expanded newborn screening” for rare genetic disorders

Rare Genetic Disease Testing

Screening Tests

Testing newborns for possible genetic disorders

Newborn Mass Screening

Provided free to all babies born in Japan
(e.g., phenylketonuria, congenital hypothyroidism)

Extended newborn screening

Fee-based tests provided to applicants
Diseases excluded from mass screening
(e.g., Pompe disease,mucopolysaccharidosis)

Genetic Tests
(definite diagnosis)

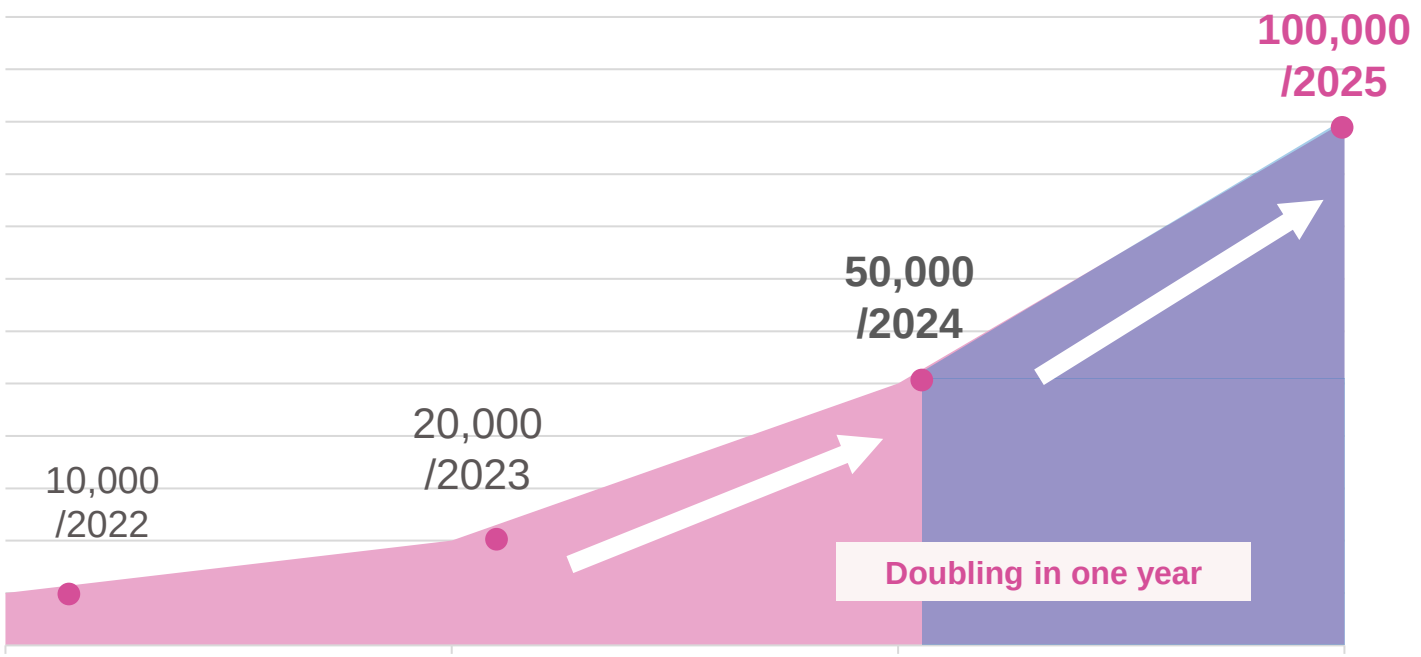
If screening tests indicate possible disease, determine the presence or absence of disease

Biomarker Tests
(monitoring treatment efficacy)

Provide data to determine the effectiveness and improvement of treatment after the start of treatment

Expanded Newborn Screening of ACRL

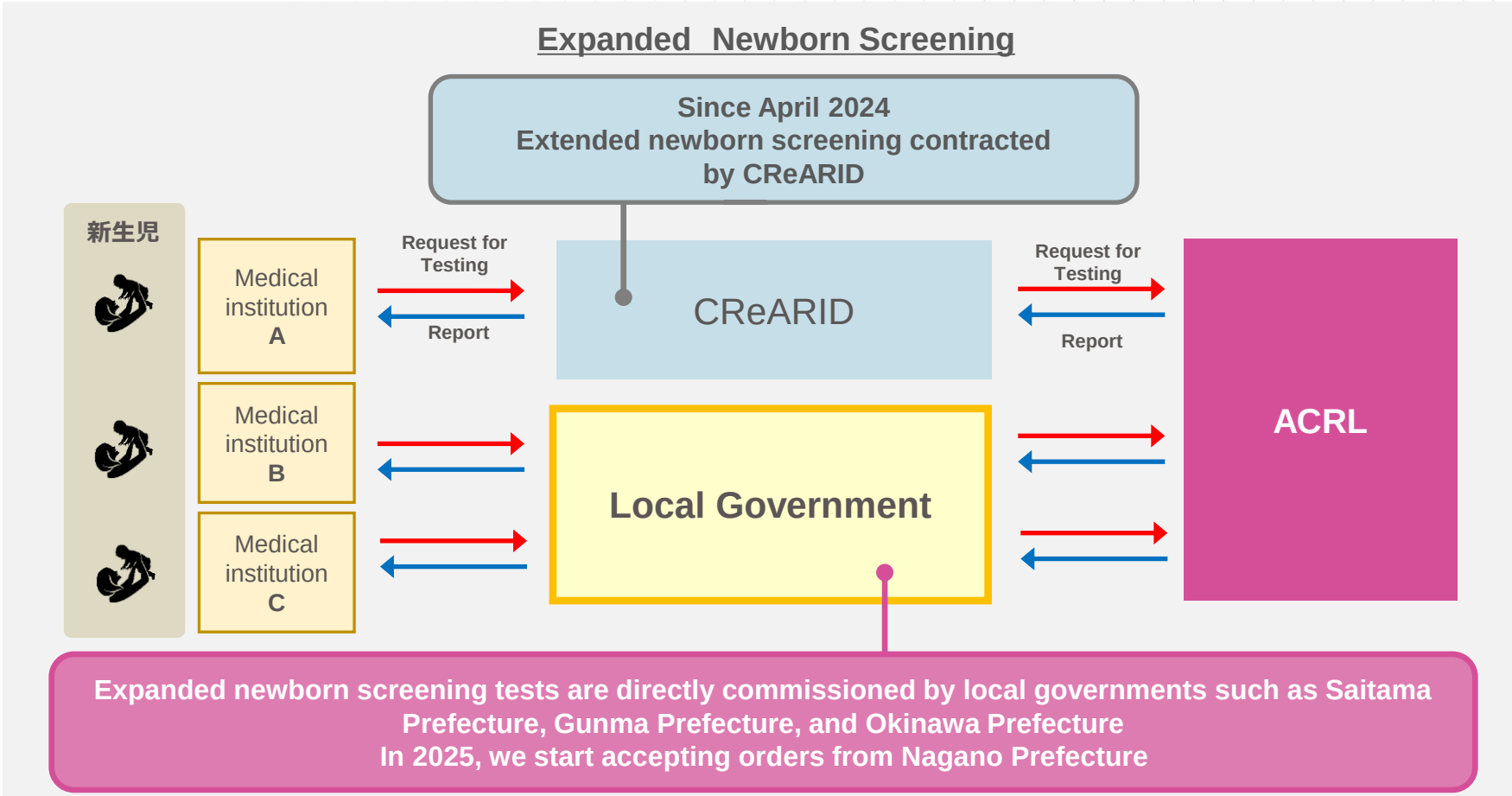
Number of contracts continues to increase



Trying for expanding the number of contractors and diseases tested

Accepting contracts for expanded newborn screening from local governments

Contract expanded newborn screening for rare genetic disorders for which early diagnosis and treatment are extremely effective, directly from local governments



Commenced genetic testing services

Rare Genetic Disease Testing

Screening Tests

Testing newborns for possible genetic disorders

Genetic Tests
(definite diagnosis)

If screening tests indicate possible disease, determine the presence or absence of disease

Biomarker Tests
(monitoring treatment efficacy)

Provide data to determine the effectiveness and improvement of treatment after the start of treatment

Commenced commissioning of definitive testing for target diseases in conjunction with the launch of Zokinvy

Newborn Mass Screening

Provided free to all babies born in Japan
(e.g., phenylketonuria, congenital hypothyroidism)

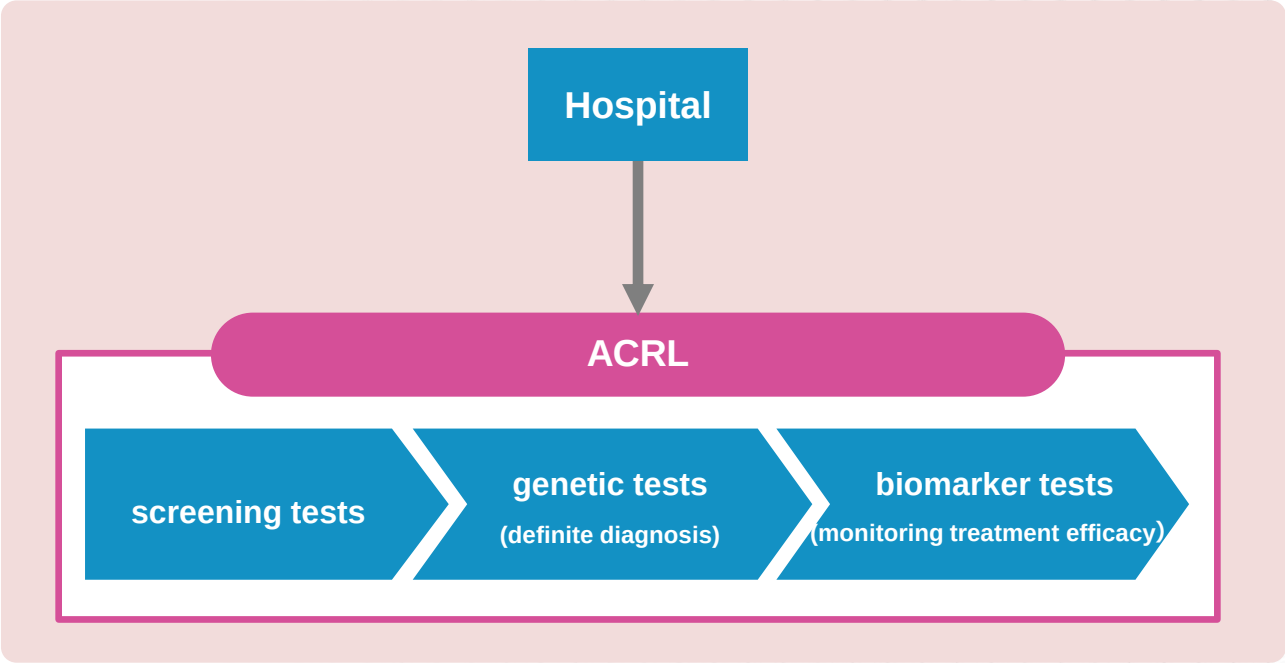
Extended newborn screening

Fee-based tests provided to applicants
Diseases excluded from mass screening
(e.g., Pompe disease,mucopolysaccharidosis)

Reduce the burden on medical professionals involved in rare genetic diseases

The only one-stop testing for rare genetic disorders in Japan

While there are few laboratories that perform all the tests for rare genetic diseases due to lack of profitability, ACRL has the necessary systems and functions for rare genetic disease testing, including "screening tests," "genetic tests" for definitive diagnosis of diseases, and "biomarker tests" for monitoring treatment efficacy, without requiring patients and doctors to request multiple laboratories.



03

Other developed products, collaboration with partner companies

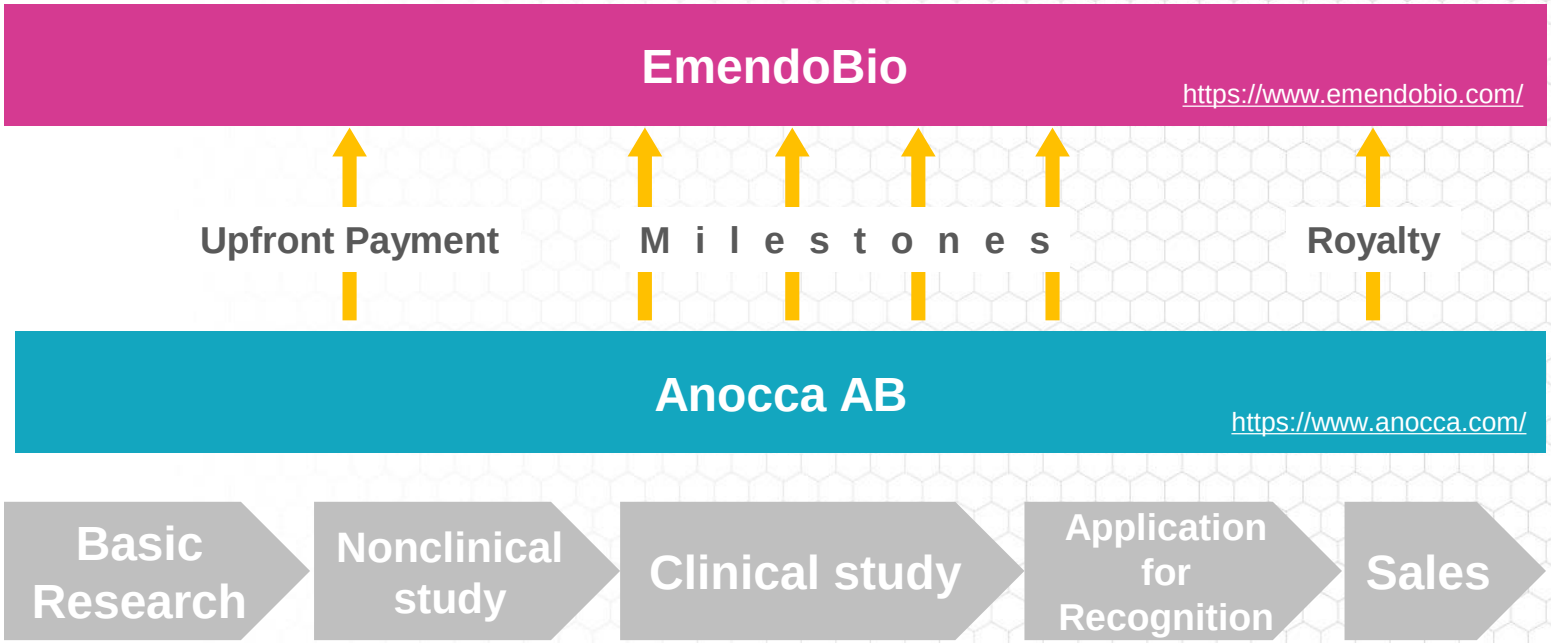
4	EmendoBio Genome Editing
5	Phase 2 Clinical Trial of Tie2 Receptor Agonist

Licensing Agreement Details.

March 14, 2024

License Agreement with Emendo and Anocca of Sweden

（Granted to Anocca a non-exclusive license to use Emendo's OMNI nuclease）



Treatment with TCR-T cells

Anocca to develop TCR-T cell therapy targeting KRAS protein mutations in solid cancer using OMNI nuclease

EmendoBio

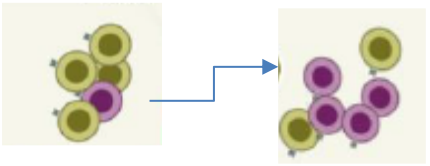
OMNI nuclease for more accurate and efficient genome editing than Cas9

emendobio

+

Anocca AB

Technology to select and culture T cells expressing TCRs that recognize specific antigens

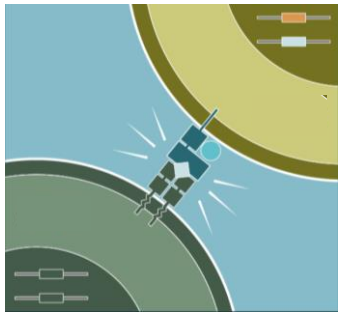


anocca

Generation of TCR-T cells that recognize cancer cell-specific antigens

Anocca has a library of TCR receptors specific to various cancers

Anocca uses OMNI nuclease to incorporate the TCR receptor gene of the target cancer into the patient's T cells



Immunotherapy

A therapy in which a patient's T cells are harvested, modified to fight cancer, and returned to the body

Normal tissues are less likely to be attacked
because they are based on the patient's immune cells

Blood cancer
Occurs due to abnormalities in hematopoietic tissue and increases without clumping
(Leukemia, malignant lymphoma, myeloma, etc)

CAR-T cell therapy

CAR-T cell therapy is a treatment in which chimeric antigen receptors are introduced into T cells using genetic modification technology, grown outside the body, and infused into the patient.
Although CAR-T has been used with great success in blood cancers, there are challenges in applying it to solid tumors because it can only recognize cell surface antigens.

Solid cancer
Cancer cells form in clusters and grow in clumps
(Pancreatic cancer, lung cancer, etc.)

Anocca AB **EmendoBio**

TCR-T cell therapy

Artificial T cells expressing TCR receptors that recognize cancer-related antigens present in cells (TCR-T cells) can be applied to solid tumors, unlike CAR-T aimed at hematologic tumors, such as CD19 CAR-T.
It has attracted significant attention as a treatment for inoperable and refractory solid tumors with minimal side effects.

Collaboration with Stanford University

Begins joint research with Stanford University on novel cancer therapies using OMNI nuclease

EmendoBio

OMNI nuclease for more accurate and efficient genome editing than Cas9

emendobio

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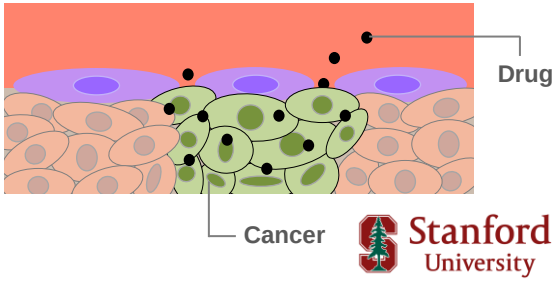
Stanford University

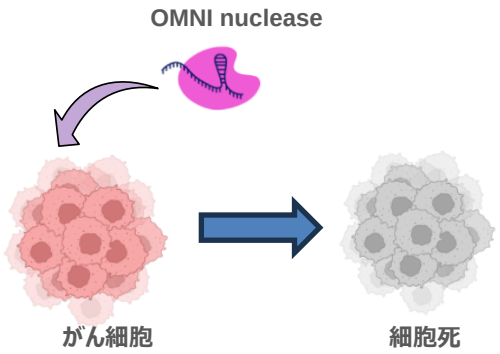
Technology to deliver drugs specifically to cancer tissue



Stanford University

Development of Novel Cancer Genome Editing Therapies Targeting Breast Cancer

Stanford University is researching advanced cancer tissue-specific drug delivery technologies, cancer immunotherapy, and more.
We will use OMNI nuclease to perform genome editing to decrease the resistance of cancer cells to therapy and develop therapies that kill only the cancer cells.



4	EmendoBio Genome Editing
5	Phase 2 Clinical Trial of Tie2 Receptor Agonist

Tie-2 Receptor Agonist

Phase 2 clinical trials are underway in the U.S.

Phase 2 clinical trials underway for acute respiratory distress syndrome (ARDS), including viral and bacterial pneumonia such as novel coronavirus and influenza

Independent Data and Safety Monitoring Board (IDSMB) has issued a positive safety evaluation for the ongoing Phase II clinical trial in the U.S.

In clinical trials in the U.S., the IDSMB, a third-party organization independent of the study sponsor, monitors the safe conduct of clinical trials, and the IDSMB conducts safety assessments as needed during the conduct of clinical trials.

Additional Medical Institutions to Conduct Clinical Trials Toward Target Patient Enrollment in Phase II Clinical Trials
Strengthening collaboration with medical institutions based on our clinical trial expertise
Accelerate clinical development

Aiming to complete registration by the 2’nd half of FY2025

AV-001, a Tie2 receptor agonist, is being developed in collaboration with Canadian biopharmaceutical company Vasomune Therapeutics, Inc.

Leading Global in Gene Medicine

AnGes's website
https://www.anges.co.jp/en/