AnGes

# FY2025 Q2 Financial Results Materials

August 2025

- **This document contains forward-looking statements, including our current outlook, forecasts, goals, and plans. These statements are based on information currently available and the judgment of our management. Therefore, actual results may differ significantly from these forecasts due to various risks and uncertainties. Please do not rely solely on these forward-looking statements. We are under no obligation to revise or update these statements in light of new information or future events.**

- **Risks and uncertainties include changes in the economic environment, progress in R&D, regulatory approvals, and changes in laws and regulations in Japan and abroad.**

## 1 Overview of FY2025 Q2 Financial Results

## 2 FY2025 Q2 Topics

① HGF Gene Therapy Product
② NF-κB Decoy Oligo DNA
③ Tie2 Receptor Agonist AV-001
④ Zokinvy
⑤ ACRL
⑥ EmendoBio Genome Editing Technology

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# Overview of FY2025 Q2 Financial Results

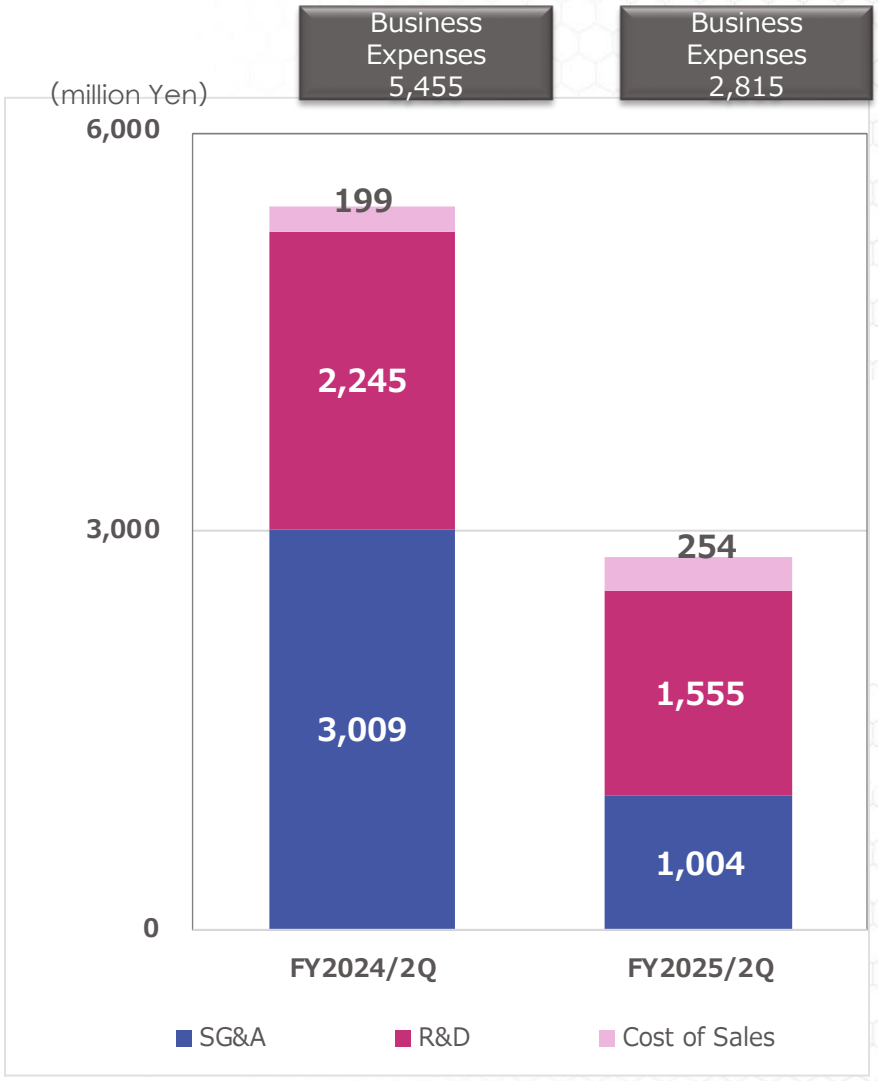
# FY2025 Q2 Consolidated Financial Results

(Million Yen)

| | FY2024/2Q | **FY2025/2Q** | Increase /Decrease |
|---|---|---|---|
| **Revenues** | 347 | **414** | 67 |
| **Business Expenses** | 5,455 | **2,815** | △2,640 |
| **Operating Profit** | △5,107 | **△2,400** | 2,707 |
| **Non-operating income/expenses** | 1,917 | **△1,498** | △3,415 |
| **Ordinary Profit** | △3,190 | **△3,898** | △708 |
| **Extraordinary income/Expenses** | △227 | **0** | 227 |
| **Profit** | △3,500 | **△3,966** | △465 |

**・The increase in business revenue for the current period is due to the steady growth in the number of commissioned testing services.**
**・Business expenses decreased due to the elimination of goodwill amortization, resulting in a significant improvement in operating profit/loss.**

# Breakdown of Business Expenses




**Cost of sales: 254 million yen**

(YoY +55million yen)
Increase in contracted testing service 162M yen (YoY+70M yen)
Cost of goods sold by Zokinvy 92M yen (YoY△7M yen)

**R&D expenses: 1,555 million yen**

(YoY △689million yen)
research material costs YoY△529M yen
Emendo's restructuring YoY△106M yen
Outsourcing expenses YoY△64M yen

**SG&A expenses: 1,004 million yen**

(YoY △2,005million yen)
①Emendo-related compensation YoY△176M yen
②Paid service fees YoY△86M yen
③Amortization of goodwill YoY△1,668M yen

# Consolidated Balance Sheet Highlights

(million yen)

| | FY2024 | FY2025/2Q | Increase /Decrease |
|---|---|---|---|
| **Current assets** | 3,542 | **4,728** | 1,186 |
| **Cash and deposits** | 1,707 | **2,908** | 1,200 |
| **Non-current assets** | 1,125 | **1,057** | △68 |
| **Total assets** | 4,668 | **5,786** | 1,117 |
| **Liabilities** | 2,512 | **2,384** | △127 |
| **Net assets** | 2,156 | **3,402** | 1,245 |

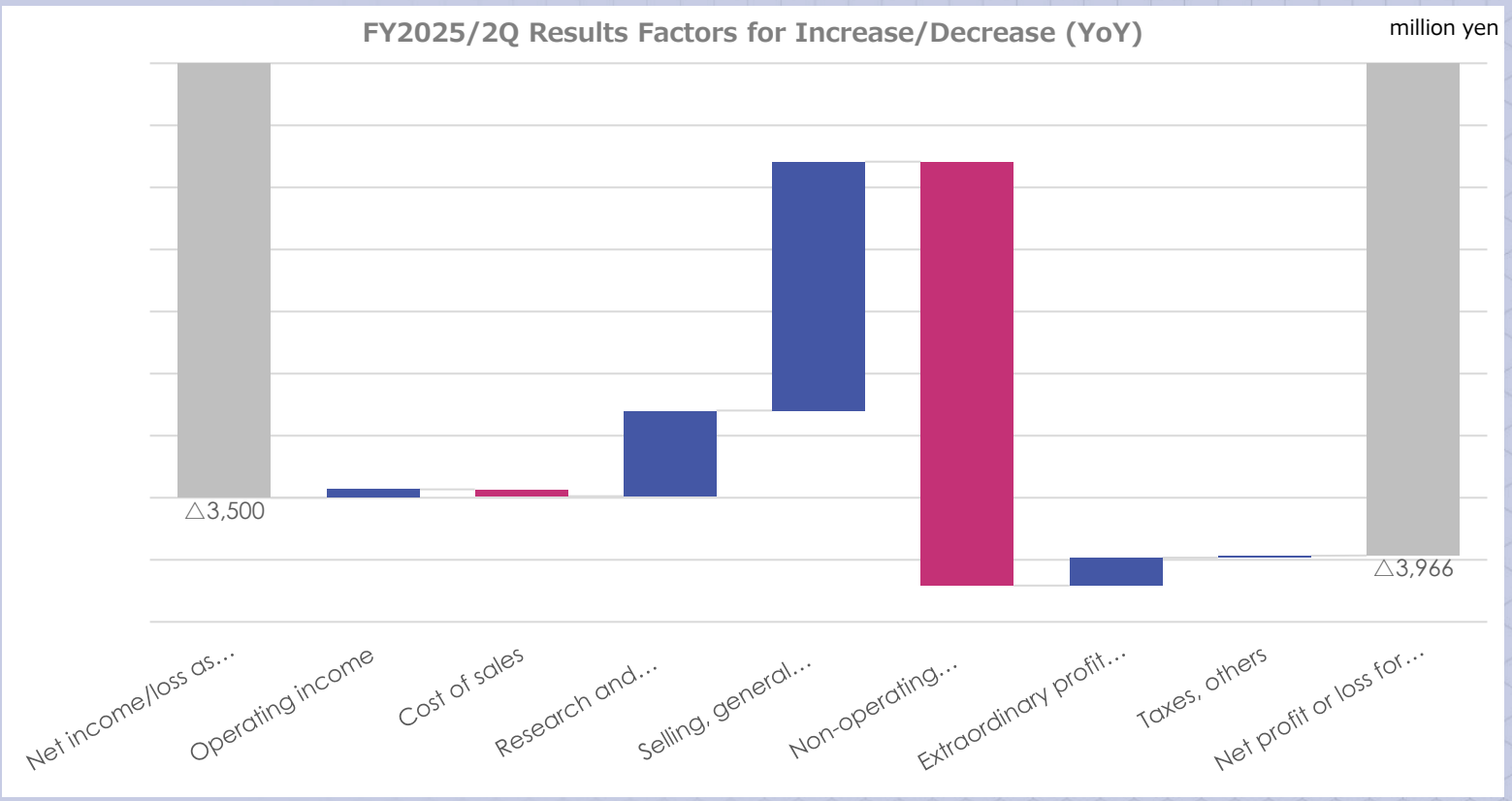
**Current Assets**

■ Cash and deposits: 2,908 M yen
(up 1,200 M yen from FY2024)
→ 3,764 M yen raised through the 45th series of stock acquisition rights
■ Merchandise: 179 M yen
(down 45 M yen from FY2024)
■ Raw Materials and Supplies: 1,265 M yen
(up 61 M yen from FY2024)

**Net Assets**

■ Capital stock / Capital surplus: +1,895 M yen
■ Retained earnings (due to net loss): -3,966M yen

# Factors Affecting Net Profit/Loss for the Period

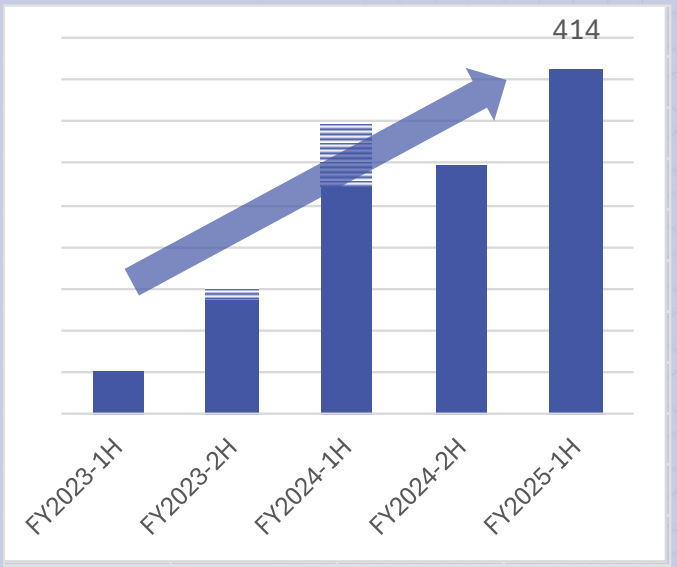



- SG&A expenses decreased due to the elimination of goodwill amortization
- Non-operating loss was significantly impacted by foreign exchange effects

# Performance Trends

## Business Revenue

million yen

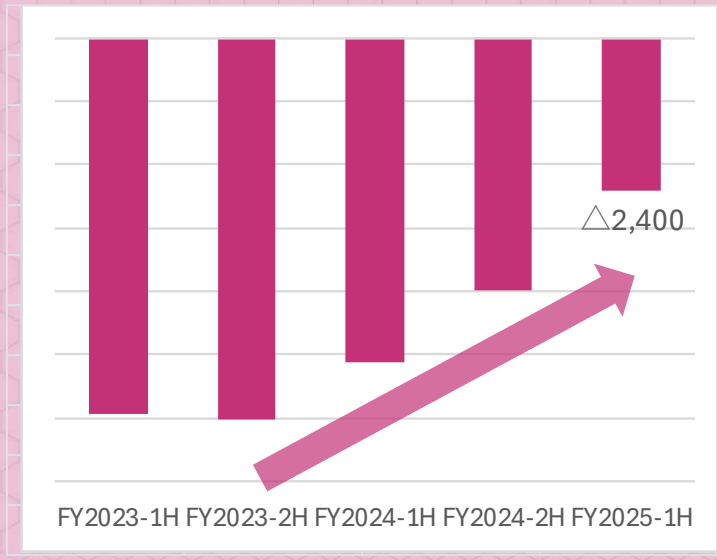



**Operating revenue increased**

・Increase in commissioned expanded newborn screening tests at ACRL
・Steady sales of Zokinvy

## Operating Profit/Loss

million yen




**Operating profit/loss improved**

・Cost reductions due to Emendo's business restructuring
・Elimination of goodwill amortization contributed to improved profitability

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# FY2025 Q2 Topics

# Status of Our Development Projects

| Project | Region | Licensing /Partner | Formulation | Indication | Basic Research | Non-clinical trials | Clinical trials | | | Application Review | Approval |
|---|---|---|---|---|---|---|---|---|---|---|---|
| | | | | | | | Phase I | Phase II | Phase III | | |
| HGF gene therapy product (bepelminogen perplasmid) | Japan | – | Injectable | Chronic arterial occlusion | | | | | | Under consideration while monitoring progress in the United States | |
| | United States | – | Injectable | Chronic Limb-Threatening Ischemia (CLTI) | | | | | | | Towards BLA Preparation started |
| | Israel Turkey | Kamada Er-Kim | Injectable | Chronic arterial occlusive disease | | | | | | | |
| NF-κB decoy oligodeoxynucleotide | Japan | – | Injectable | Chronic discogenic Low back pain | | | | | | | |
| DNA vaccine | Australia | – | Injectable | Hypertension | | | | | | | |
| Tie2 receptor agonist | United States | Vasomune | Injectable | Acute respiratory distress Syndrome (ARDS) | | | | | | | |

# FY2025 Q2 Topics

| | |
|---|---|
| 1 | Progress of HGF Gene Therapy Product |
| 2 | Progress of NF-κB Decoy Oligo DNA |
| 3 | Progress of Tie2 Receptor Agonist |
| 4 | Sales of Zokinvy |
| 5 | Commissioned Testing at ACRL |
| 6 | EmendoBio Genome Editing Technology |

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# Development and Commercial Strategy in the U.S.

**The HGF gene therapy product has completed clinical trials in the U.S. and is now preparing for Biologics License Application (BLA) submission, aiming for early approval**

General process for application and approval


Process of HGF Application and Approval in U.S.

# Review of HGF Gene Therapy Product Development

## <Japan>

**November 2014**
The conditional and time-limited approval system was implemented

**January 2018**
Utilizing the conditional and time-limited approval system, an application for manufacturing and marketing approval was submitted

**March 2019**
Conditional and time-limited approval was obtained

**September 2019**
Sales began by Mitsubishi Tanabe Pharma

**May 2023**
Application for formal manufacturing and marketing approval was submitted

**June 2024**
Withdrawal of the application for formal approval※

※Due to the withdrawal of the approval application for lifting the conditions, the domestic sales of Collategen have ended.

**Priority was given to the application for approval in U.S., and future policies will be considered while monitoring its progress**

## <United States>

**2014年**

**2014～2016**
**Global Phase 3 Trial**
Targeting patients at high risk of limb amputation in 15 countries (U.S., Europe, South America), but discontinued due to insufficient enrollment.

**2019年**

**June 2019**
**Publication of new global treatment guidelines (GVG)**

**February 2020**
**Start of Phase 2b trial enrollment**

**2023年**

**March 2023**
**Completion of patient enrollment**

**2024年**

**June 2024**
**Favorable results confirmed from Phase IIb trial targeting patients with lower risk of limb amputation**

**September 2024**
**Designated as Breakthrough Therapy by FDA**

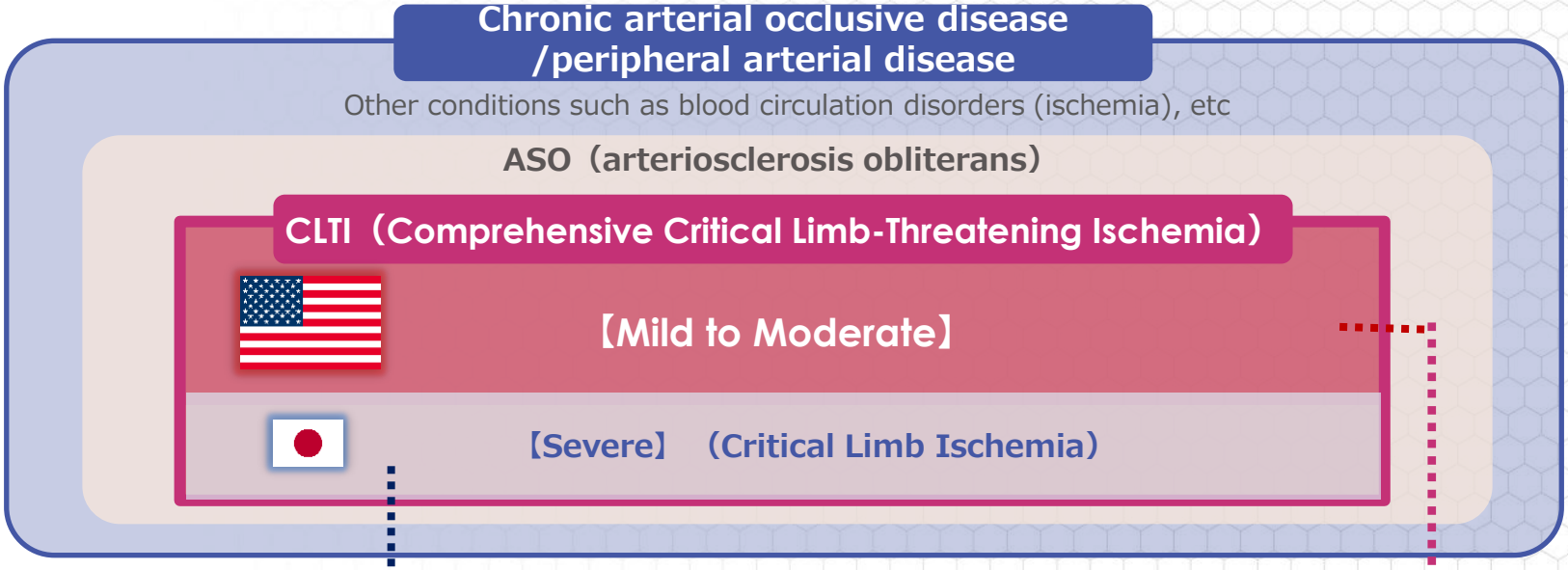
**November 2024**
**Results presented at AHA Scientific Sessions**

**2025年**

**2025年８月**
**The clinical trial will be completed, and preparations will be made toward applying for approval of the Biologics License Application (BLA)**

# Scope of diseases in clinical trials in Japan and the U.S.

**Based on advice from the lead investigator that "it is important to treat patients before the condition becomes severe," the U.S. Phase 2b clinical trial targeted patients with mild to moderate CLTI (Chronic Limb-Threatening Ischemia)**




**Target Patients in Japanese Clinical Trials**

With ulcers due to occlusive arteriosclerosis
The patients were those with severe lower limb ischemia

**Target Patients in U.S. Clinical Trials**

Based on advice from clinical trial supervisors
The clinical trial targeted patients with mild to moderate CLTI.
A clinical trial was conducted

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# NF-κB Decoy Oligo DNA

**In 2025, a paper was published in *The SPINE JOURNAL*, issued by the North American Spine Society (NASS), detailing the results of a Phase 1 clinical trial conducted in the U.S. since February 2018 targeting patients with discogenic low back pain**

**【Results of the Phase 1b Clinical Trial of NF-κB Decoy Oligo DNA】**

- No decline in neurological, sensory, or motor function was observed in any group (placebo or AMG0103 at 0.3mg, 3mg, or 10mg) over the one-year observation period. No serious adverse events occurred, and no safety concerns were identified.
- AMG0103 showed dose-dependent analgesic effects, with the highest dose (10mg) resulting in an average 77% reduction in pain one year after administration.
- While disc height decreased in the placebo group, it increased in the 10mg group, suggesting morphological improvement.
- Improvements were also observed in patient satisfaction (PGIC) and daily activity scores (RMDQ, ODI). No patients in the 10mg group required additional pain medication during the trial, indicating sustained analgesic effects.

**・77% of patients experienced pain relief, and half had near-complete pain resolution**

**・Disc height improvement suggests potential recovery of damaged discs**

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# Tie2 Receptor Agonist (AV-001)

**As a treatment for acute respiratory distress syndrome (ARDS)**

**Designated as Fast Track by the US FDA**

## What is Fast Track?

A system that prioritizes the review of drugs for serious diseases or unmet medical needs

**To deliver promising drugs to patients more quickly**

## Benefits of Fast Track

**More frequent meetings and communication with the FDA**

**Accelerated approval and priority review**

**Allows for rolling submission and review before all data is finalized**

Due to the short influenza season in North America,

**Complete target patient enrollment by the second half of 2025**

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# About Zokinvy

## Sales began on May 27, 2024

- 2022 May: Exclusive distribution agreement with Eiger BioPharmaceuticals for Japan
- 2022 May: Orphan drug Designation application
- 2023 March: Orphan drug Receive designation
- 2023 May: Application for manufacturing and marketing approval of 'Zokinvy' in Japan
- 2024 January: Manufacturing and marketing approval obtained in Japan
- 2024 May: Sales launched in Japan

## Zokinvy (lonafarnib)


**【Drug Price】**

Left: ¥91,796.40 per capsule 50mg (per capsule)
Right: ¥136,544.00 per capsule 75mg (per capsule)

**【Indications, efficacy, or performance】**

Hutchinson-Gilford Progeria Syndrome (HGPS) and processing-deficient progeroid laminopathies

**【Dosage and Administration】**

Initial dose: 115 mg/m² body surface area, orally twice daily with or immediately after meals
After 4 months: Maintenance dose of 150 mg/m², orally twice daily with or immediately after meals
Dose adjustments may be made based on patient condition

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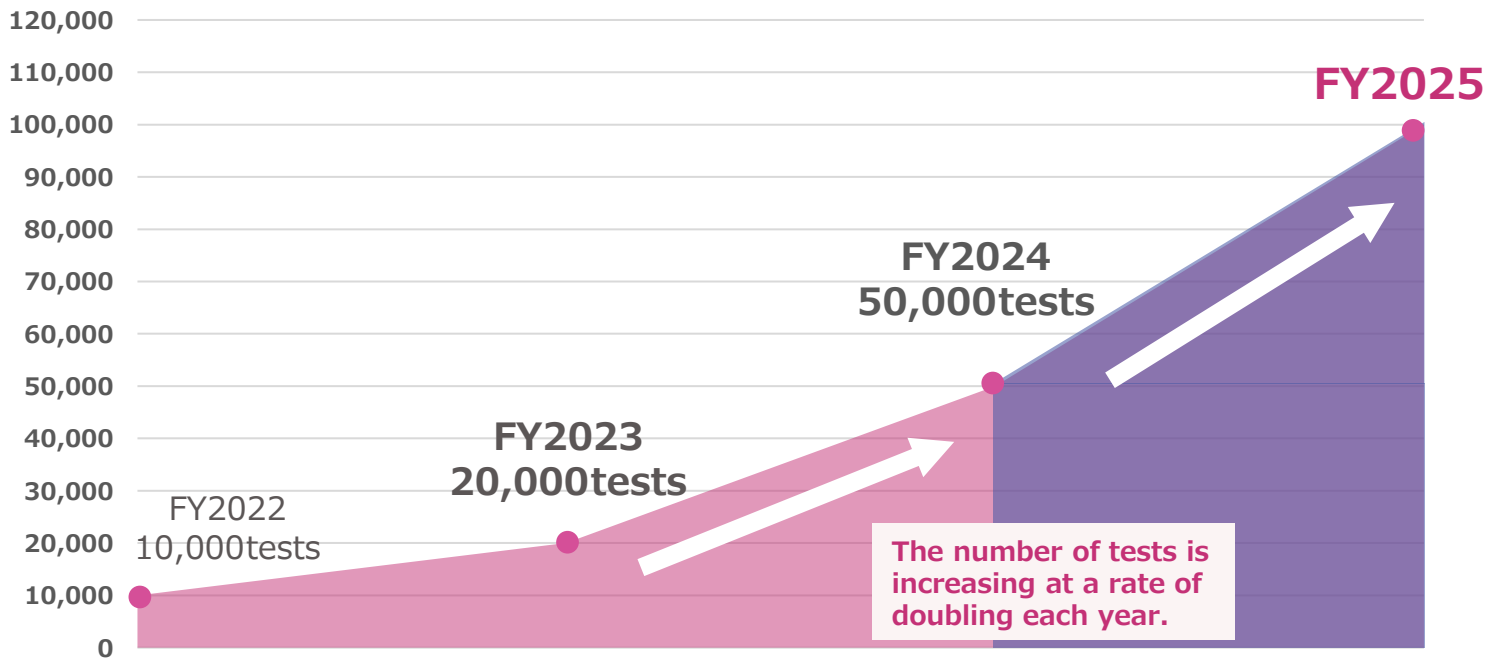
# Status of Expanded Newborn Screening at ACRL

**The number of expanded newborn screening tests continues to increase**
**In FY2024, approximately 50,000 tests were commissioned**
**In FY2025, the number of commissioned tests is expected to double**




**We continue to expand both the number of clients and the range of target diseases**

# Biomarker testing services are being prepared

## Rare genetic disease testing

### Screening test

Test to check whether newborns have a possibility of hereditary diseases

#### Newborn Mass Screening (Public Mass)

・Provided free of charge to all newborns in Japan (funded by local governments)
・For congenital metabolic disorders (such as phenylketonuria), and endocrine diseases (such as congenital hypothyroidism)

### Genetic testing
(Definitive test)

If the screening test suggests the possibility of a disease, the presence or absence of the disease is confirmed

From May 2024

#### Expanded newborn screening

・Conducted for those who request it, for a fee
・Diseases not covered by mass screening (such as Pompe disease, mucopolysaccharidosis, etc.)

From April 2021

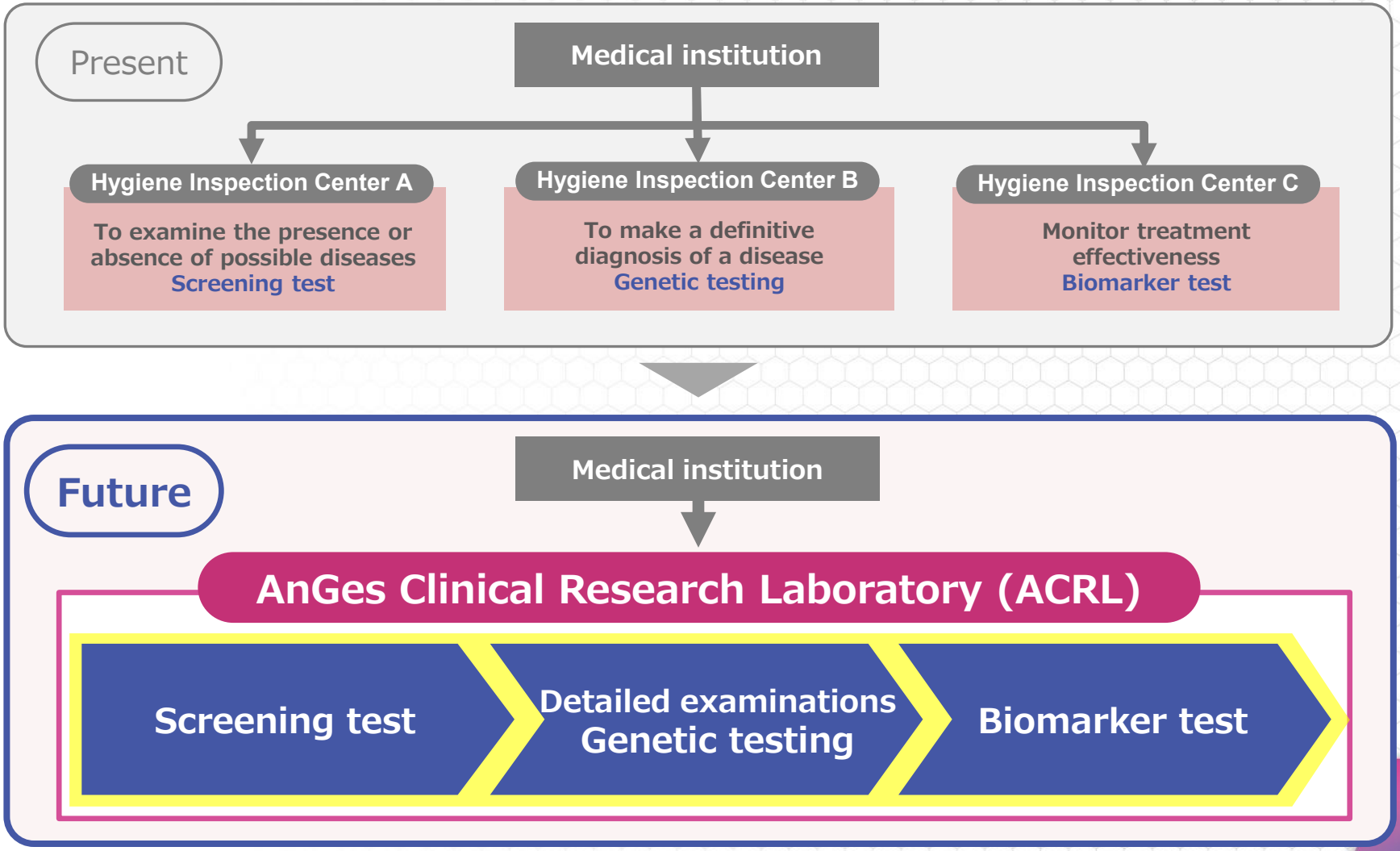
### Biomarker testing
(Monitoring of treatment efficacy)

If the screening test suggests the possibility of a disease, the presence or absence of the disease is confirmed

Preparations are underway to start receiving orders this fall

# One-Stop Testing Services for Rare Genetic Diseases

**At ACRL, we aim to build a system that can provide comprehensive testing services for rare genetic diseases.**



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# Joint research with Stanford University

## Using the OMNI nuclease developed by EmendoBio
## Launch of joint research and development of a novel cancer therapy with Stanford University

**EmendoBio**

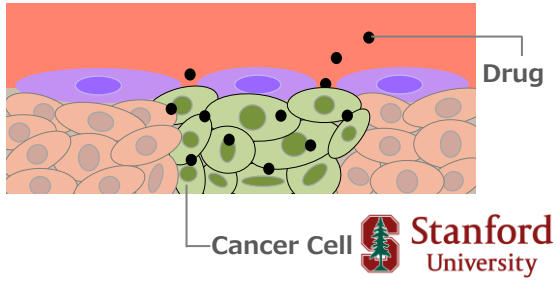
More precise than conventional Cas9
OMNI nuclease enables more efficient genome editing

emendobio

+

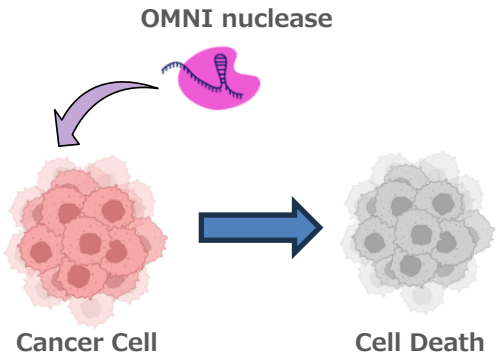
**Stanford University**

Technology for delivering drugs specifically to cancer tissues
Drug delivery technology




### Development of a novel genome editing therapy targeting breast cancer

At Stanford University, advanced research has been conducted in the development of new cancer treatments such as technologies for cancer tissue-specific drug delivery, cancer radiotherapy, and cancer immunotherapy. By using Emendo's OMNI nuclease to edit the genome specifically in cancer cells, we aim to develop a treatment method that kills only cancer cells by reducing their therapeutic resistance.



# Leading Global in Gene Medicine

**AnGes, Inc.**
**https://www.anges.co.jp/en**