AnGes

FY2026-3Q Financial Results Materials

November 2026

Major Business Highlights for FY2026-3Q

【HGF Gene Therapy Product】：Preparing for BLA in the US

US：Preparing for Biologics License Application (BLA)

Rolling Submission (initial document submission) scheduled to begin in Q4

→Preparation in coordination with the FDA

JP：Future actions are under consideration based on progress in the US

【NF-κB Decoy Oligonucleotide DNA】：P2Clinical Trial Ongoing in Japan

Results from the P1 clinical trial in the US were published in *The SPINE Journal*, the official journal of the North American Spine Society.

→Aiming to complete registration of the planned number of cases by fiscal year 2026

【Tie2 Receptor Agonist】：Completion of Phase 2 clinical trial registration for ARDS in the US

Completed patient enrollment for the Phase 2 clinical trial in the United States

→ Preliminary test results are expected to be released in Q1/ 2027

Progress in Physician-Initiated Trial Evaluating Reduction of Cognitive Impairment in Hemodialysis Patients

Secured support from the National Research Organization of Canada Industrial Technology Support Program for subcutaneous injection formulation development

Major Business Highlights for FY2026-3Q

【Zokinvy】：Revising Sales Outlook

【ACRL】：Contracting for expanded newborn screening remains stable

- Expanded Newborn Screening
  The number of contracted diseases has expanded from 9 to 14, and the number of contracts has remained stable
- Establishment of a Comprehensive Testing Framework
  Expanding the range of diseases covered by genetic testing
  →In addition to HGPS and PDPL, seven diseases have been added to the expanded newborn screening target
  Research Utilizing Biomarker Testing for Secondary Screening Published

【EmendoBio】：Promoting research and business development of OMNI nuclease

Establishing a system to advance research and development activities and exports in the US

Promoting joint research with Stanford University on new cancer genomic therapies

Considering opening a new research and development center in the US

Anocca's license revenue is expected to shift to next fiscal year

Performance for FY2026-3Q

Although business revenue increased, business expenses increased due to HGF application preparation costs

(Unit：Million Yen)

	FY2025-1Q	FY2026-3Q	Change
Revenue	646	**751**	104
Operating Expenses	4,188	**5,776**	1,587
Operating Profit(Loss)	-3,542	**-5,025**	-1,482
Non Operating Income(Expenses)	-1,073	**92**	1,166
Ordinary Profit(Loss)	-4,615	**-4,932**	-316
Extraordinary Income(Expenses)	0	**-25**	-25
Net Profit(Loss)	-4,688	**-5,081**	-392

Revenue（YoY +16.2%）

- Zokinvi's sales of 328 million yen (YoY +51.6%)
- Screening tests at ACRL amounted to 422 million yen (YoY +2.2%)

Operating Expenses（YoY +37.9%）

- Cost of Sales: 388 million yen (YoY +1 million yen)
  Rising cost of goods sold at Zokinvi
- Research and development expenses: 3,870 million yen (YoY +1,495 million yen)
  Mainly the increase in costs related to preparing HGF applications
- SG&A expenses: 1,517 million yen (YoY +91 million yen)
  Mainly increased financing-related expenses

Non Operating Income

- Foreign exchange gain of 165 million yen from revaluation of loans to EmendoBio

Special Gains and Losses

- Profit from investment securities sales: 11 million yen
- Impairment of EmendoBio Right-of-Use Assets Decreased by 41 million yen

Consolidated Balance Sheet Highlights

(Unit：Million Yen)

	Dec-2025	September-2026	Change
Current Assets	4,386	2,470	-1,915
Cash and Deposits	1,882	233	-1,648
Non-Current Assets	1,019	1,001	-17
Total Assets	5,405	3,472	-1,933
Liabilities	2,329	4,830	2,500
Net Assets	3,076	-1,357	-4,433

Curent Assets

531 million yen through the 46th stock acquisition rights
647 million yen through bond issuance
241 million yen through the 47th and 48th stock acquisition rights
2,000 million yen through bonds with stock acquisition rights
Raised 100 million yen through borrowings
Due to expenditures on business expenses, etc.: -1,648 million yen

Net Assets

By exercising the 46th and 47th stock acquisition rights
Capital stock and capital surplus each increased by +380 million yen
Net assets decreased by 5,081 million yen due to quarterly net loss

Revision of the FY2026 earnings outlook

(Unit：Million Yen)

	Revenue	Operating Profit/Loss	Ordinary Profit/Loss	Net Profit/Loss
FY2026 Revised plan	**1,000**	**-8,900**	**-9,000**	**-9,100**
FY2026 Initial Plan	1,330	-10,230	-10,240	-10,250
increase or decrease	-330	1,330	1,240	1,150

Business revenue declined due to the following factors:
- Revenue from EmendoBio's licensing is expected to be pushed forward to next fiscal year
- Sales outlook for Zokinvy decreased compared to the initial plan

Various profits and losses improved due to the following factors:
- Thorough cost reduction for both R&D and SG&A expenses, and review implementation timing

Appendix

HGF Gene Therapy Products

What are HGF gene therapy products?

What is Gene Therapy

Inserting specific genes into the patient's body

A treatment method that cures diseases by utilizing the action of proteins produced by these genes

What is HGF

A growth factor that promotes the proliferation of hepatocytes

In 1984, Japanese researchers discovered a growth factor in the liver, the organ with the strongest regenerative capacity

This factor is called Hepatocyte Growth Factor (HGF), and it affects not only the liver,

It has been found that it plays a major role in the formation and regeneration of various organs and tissues in the body, such as blood vessels, lymphatic vessels, and nerves

Discovered that HGF has the ability to regenerate blood vessels

In 1995, a research team from Osaka University

It was discovered that HGF has the ability to 'regenerate blood vessels,' and for ischemic diseases where blood vessels are blocked and blood flow impairs, it has achieved unprecedented 'vascular regeneration'

Development of the therapeutic drug 'HGF gene therapy' has begun

*"Creating new blood vessels" means increasing the number of new blood vessels. By regenerating (creating new ones), blood vessels are regenerated.

Mortality after 5 years of severe lower limb ischemia

What is severe lower limb ischemia

According to Dr. Armstrong from the University of Southern California in the United States,
In the United States, the five-year mortality rate for patients with severe lower limb ischemia is 57%,
and it is the second most severe disease after the 80% mortality rate of lung cancer

For severe lower limb ischemia, just like cancer treatment, Early Start of Treatment Is Important

Targeting patients with chronic arterial occlusion with a low risk of lower limb amputation
Late-stage Phase 2 clinical trial conducted

Lead physician's paper published in the American Cardiology Association's journal "Circulation: Cardiovascular Interventions"

Development History of HGF Gene Therapy Products

<Japan>

November 2014
Implementation of conditional and time-limited approval systems

January 2018
Utilizing conditional and time-limited approval systems
Application for manufacturing and marketing approval

March 2019
Obtaining conditional and time-bound approvals

September 2019
Sales started by Mitsubishi Tanabe Pharma

May 2023
Application for Official Approval for Manufacturing and Sales

June 2024
Withdrawal of application for full approval※

※With the withdrawal of the approval application to lift the condition, domestic sales of Collategen have ended

Prioritize approval applications in the United States and monitor its progress Consider future policies

<United States>

2014

2014～2016
Global Phase 3 Trial
A survey was conducted in 15 countries across the United States, Europe, and South America, focusing on the high risk of lower limb amputation.
Aimed for 500 cases among patients, but discontinued due to insufficient patient enrollment

2019

June 2019
Announcement of New Global Treatment Guidelines

February 2020
Registration for late-stage Phase II clinical trials has begun

2023

March 2023
Completion of patient enrollment for the late-stage Phase 2 clinical trial

2024

June 2024
Completion of late-stage Phase II clinical trial
Positive Results Confirmed

September 2024
Designated as Breakthrough Therapy by the FDA

November 2024
Clinical trial results presented at AHA academic sessions

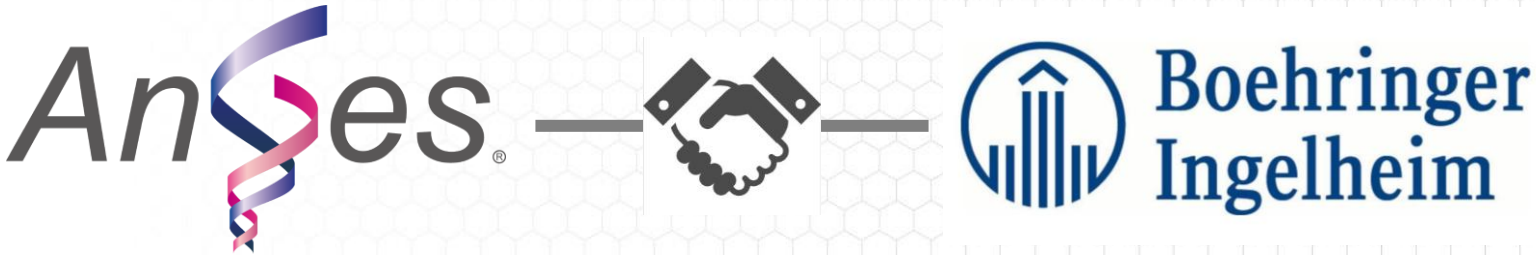
2025

August 2025
Completed clinical trials and is preparing for approval of Biologics License Application (BLA)

December 2025
Type B Clinical Meeting

Establishment of a post-market API manufacturing and supply system

It is one of the world's leading pharmaceutical and biopharmaceutical manufacturers.
With Boehringer Ingelheim Biopharmaceuticals
Started collaboration on manufacturing



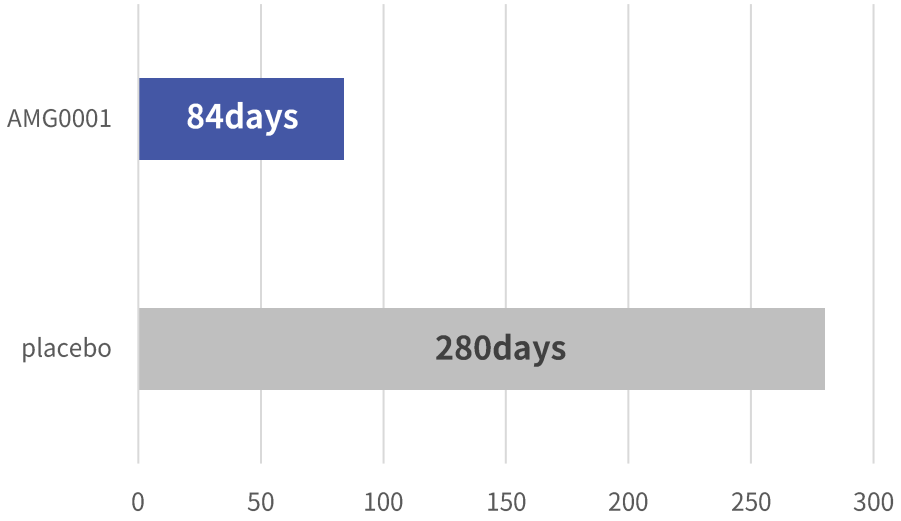
Contract signing date	**August 20, 2025**
Partners	**Boehringer Ingelheim BioXcellence™** **(One of the world's leading biopharmaceutical CDMOs)**
Content	**Contract for development and manufacturing of active pharmaceutical ingredients for HGF gene therapy products**
Meaning	**Collaboration with top players with a track record of supplying over 45 commercial products worldwide** **Ensuring the reliability of CMC information for FDA approval acquisition**

Published a paper on clinical trial results in the United States

Positive results from Phase 2 clinical trials in the United States
Published a paper in the American Heart Association (AHA) journal "Circulation: Cardiovascular Interventions"

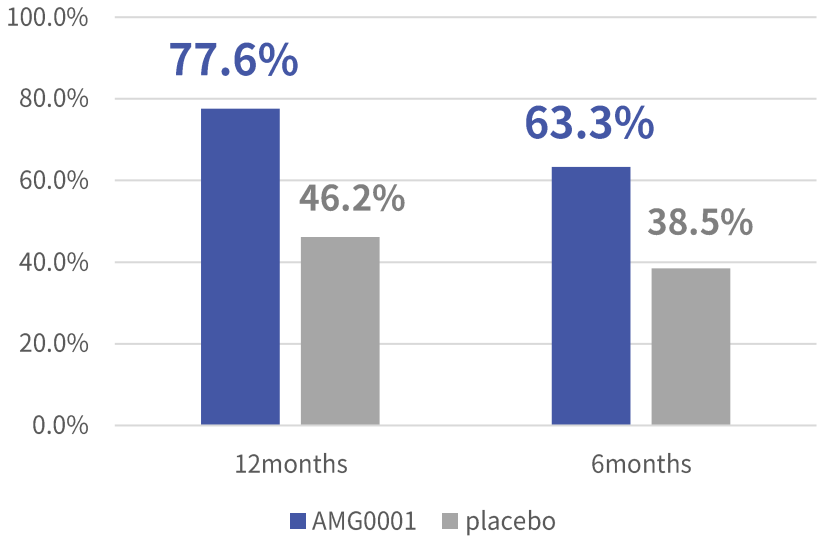
Median time to heal

Moderate CLTI patients
Significantly shortened the time until ulcer healing



Ulcer Healing Rate

Demonstrated strong efficacy in patients with moderate CLTI



※Circulation: Cardiovascular Interventions

Research and practice minimally invasive treatments for heart and vascular diseases issued by the American College of Cardiology (AHA), using catheters and similar devices
A peer-reviewed academic journal specializing in cardiovascular interventions, which is the medical field. Regarding coronary artery disease, structural heart disease, and vascular diseases.
Focusing on intervention techniques, it emphasizes original research, randomized trials, and large-scale registry studies.

FDA Biologics Application Process

Following favorable results from the U.S. Phase 2 clinical trial (P2), consultations with the FDA led to completion of the clinical trial
Once the procedures before applying for the BLA are completed, proceed to the BLA application

Typical biologics application process



Application Process for HGF Gene Therapy Products



Breakthrough Therapy
Because of this, intensive guidance from the FDA is required,
Streamlining the judging process (such as rolling submissions)
You can obtain eligibility to use the service

Rolling Submission

Instead of submitting after all areas are completed,
By submitting in order from the areas you have prepared,
Accelerate the start of the review process

Current Status of BLA Applications in the United States

Completed individual consultations (clinical and CMC) and Pre-BLA meetings with the U.S. FDA
Rolling Submission Initiated and Advance the BLA Review Process Using Priority Review

Currently — Advancing Toward BLA Submission

August 2025 Done

End of Phase 2 Meeting

In consultation with the FDA Phase 3 clinical trials are exempted,
Based on the results of the Phase 2 clinical trial, the policy to apply for BLA was decided.
Signed a commercial API supply contract with BI (Boehringer Ingelheim)

May 2026 Done

Type B Meeting

【2026.1】
Type B Clinical Meeting
Non-clinical and clinical application policy Agreement with the FDA
【2026.5】
Type B CMC Meeting
Manufacturing with Commercial Supply in Mind,
Regarding the quality control plan with the FDA Agreement

July 2026 Done

Pre-BLA Meeting

Before applying for the BLA, Final confirmation of the preparations for the BLA application

At our company, we conduct prior written Q&A sessions regarding the application materials After completing the confirmation, without holding a meeting, it is concluded

Target Completion of Rolling Submission in Q1 2027

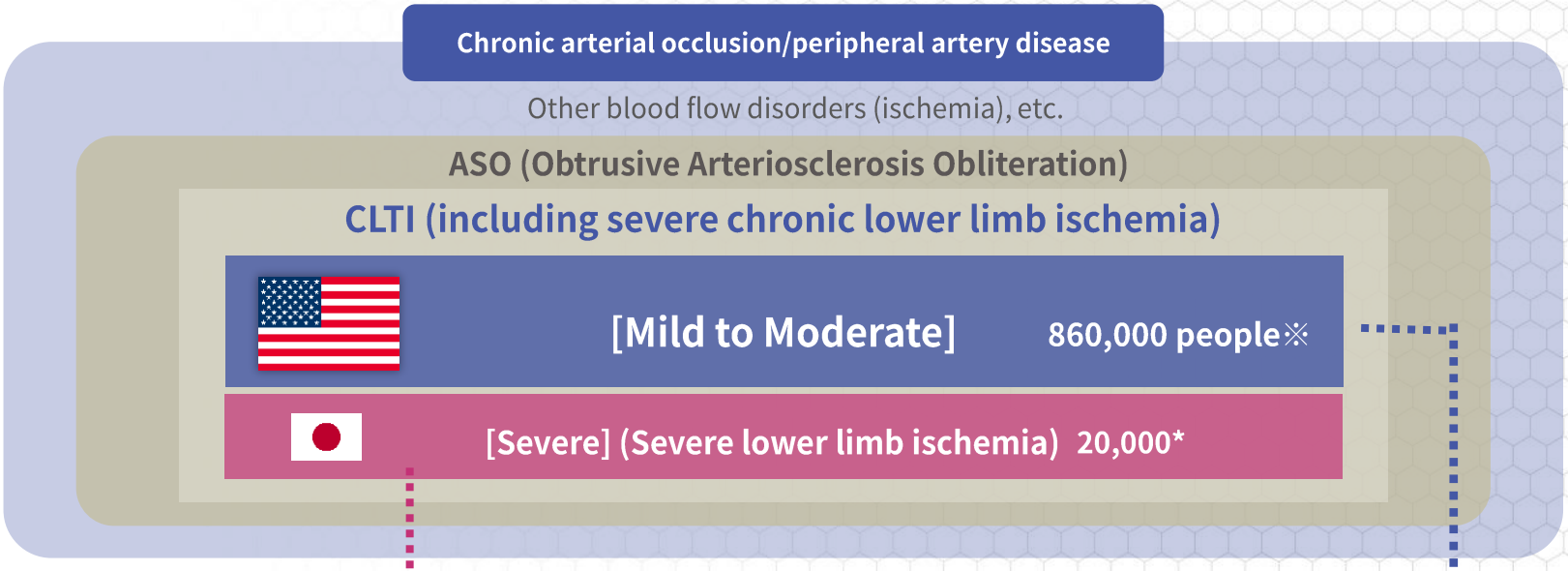
BLA

Completed application data (non-clinical
・Clinical and CMC) are submitted sequentially, and the application is proceeded while undergoing FDA review. After the final data is submitted, the BLA application is expected to be accepted as a formal BLA.

The FDA target review period is approximately six months following acceptance of the BLA.

Scope of HGF's clinical trials in Japan and the United States

Based on the clinical trial instructor's advice that 'treating patients before they become severe is important,' In the United States, late-stage Phase 2 clinical trials are being conducted targeting patients with mild to moderate CLTI



Clinical Trials in Japan
Eligible patients

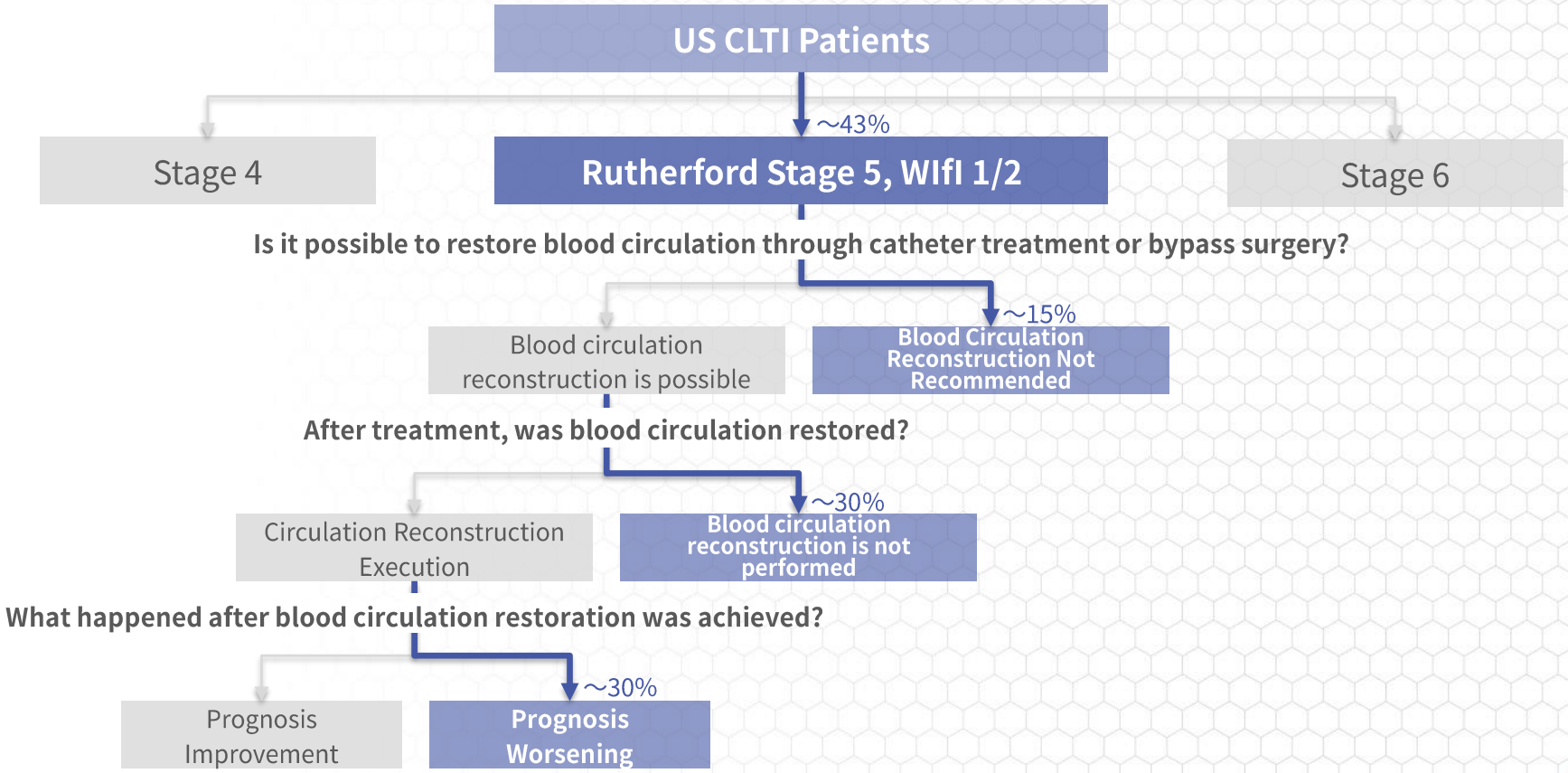
With ulcers from obstructive arteriosclerosis
It was intended for patients with severe lower limb ischemia

Clinical Trials in the US
Eligible patients

With the advice of clinical trial instructors
Targeting patients with mild to moderate CLTI
Conducting Clinical Trials

Number of patients eligible for HGF gene therapy products

Issues such as inability or failure to perform blood circulation reconstruction,
or worsening prognosis after revascularization,
Assuming administration to symptomatic patients



NF-κB decoligoDNA

Mechanism of NF-κB Decoiooligo DNA

■ Features as a Nucleic Acid Drug

NF-κB decolioligo DNA is a short DNA that is artificially created. Unlike conventional small molecule drugs or antibody drugs, it is a next-generation nucleic acid drug that can directly control the activity of genes that cause disease.

■ Mechanism of action: The "decoy" of genes

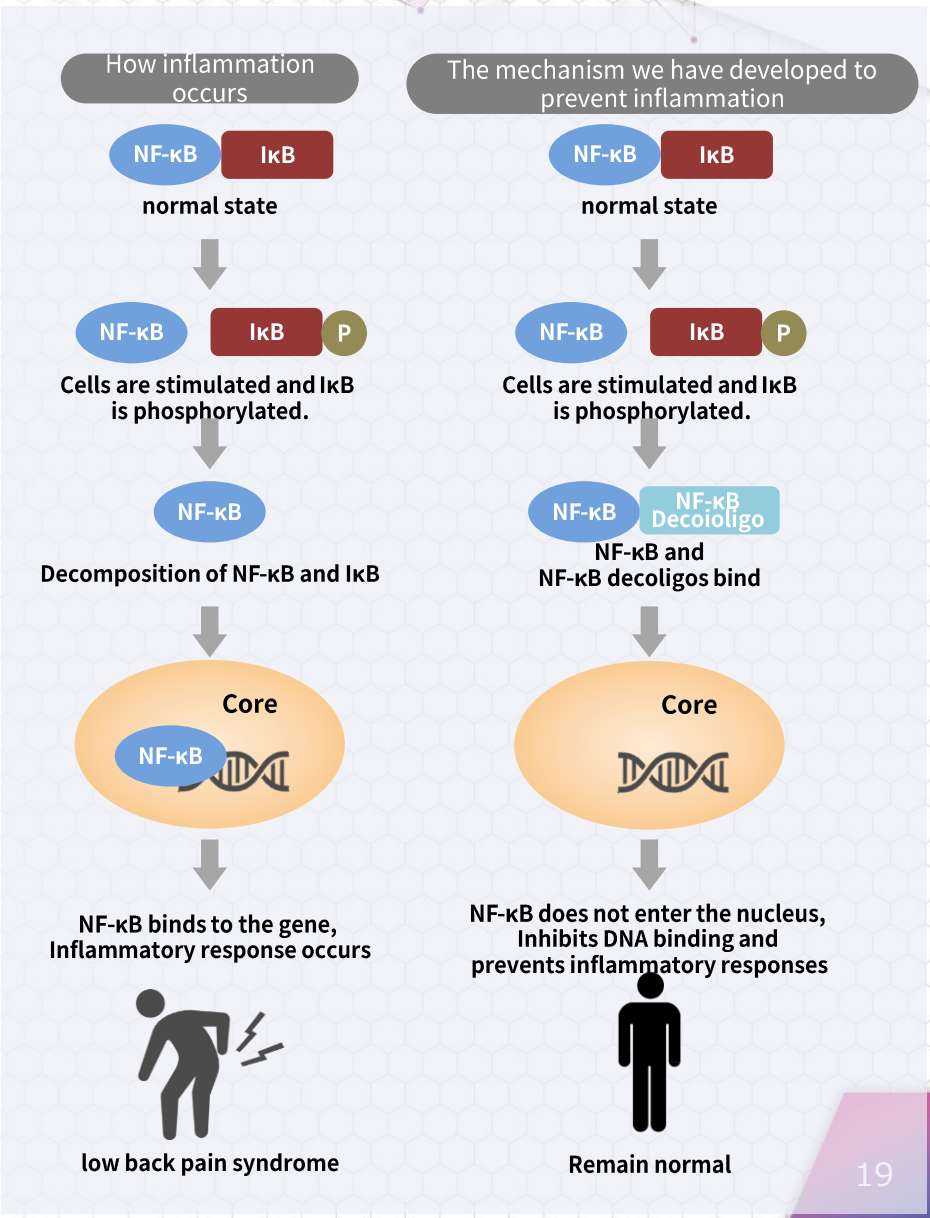
Normally, information is transmitted within the body through the flow of "DNA→ RNA→ and proteins. As the name suggests, this product functions as a "decoy" to trick gene switches.

- Stopping abnormal gene activity
- Inhibits the synthesis of pathogenic proteins

■ Indications for Chronic Intervertebral Disc Low Back Pain

Due to damage or degeneration of the intervertebral discs, NF-κB that has migrated into the nucleus binds to specific DNA sequences, leading to excessive expression of inflammatory cytokines (such as IL-6 and TNF-α).

By administering this product, NF-κB is prevented from binding to genuine DNA, fundamentally suppressing immune and inflammatory responses.



NF-κB decoligoDNA

In The SPINE JOURNAL, published by the North American Spine Society (NASS),
Conducted in the United States on patients with disc-type low back pain
Published a paper on the results of a late-stage Phase 1 clinical trial

In the 10mg group,

- **77.4% of patients** who participated in clinical trials experienced pain relief,
  Pain almost completely disappeared in half of the cases

- **The height of the intervertebral disc has improved** even before administration
  Suggests recovery of damaged discs

The currently ongoing domestic Phase 2 clinical trial

Aiming for registration completion by 2026

Tie2 Receptor Agonist (AV-001)

What is the Tie2 Receptor Agonist (AV-001)?

Tie2 receptor agonist is advancing joint development with Vasomune,
a Canadian biopharmaceutical company
Targeting diseases caused by vascular insufficiency, such as acute respiratory failure.
Phase 1 clinical trials conducted in the United States since 2020 to confirm safety and tolerability

About Vasomune

Vasomune Therapeutics, Inc. enhances the body's defense mechanisms against vascular leakage, and develops next-generation pharmaceuticals. A Canadian biopharmaceutical company founded in 2014
Vasomune is focusing on vascular normalization strategies, and has expanded its lead candidate AV-001 from basic research to clinical application.
We have been progressing with it.
Vascular dysfunction and vascular leakage can be caused by bacterial and viral pneumonia, ARDS, sepsis, hemorrhagic shock, acute kidney injury, stroke, vascular dementia, and others,
The technology has potential applications across a wide range of diseases.

What is the Tie2 receptor?

■ Tie2 Receptors
Protein receptors located inside blood vessels (endothelial cells). It mainly acts as a command center, stabilizing blood vessels and reducing leakage. Specifically:
- Maintains vascular barrier function
- Strengthening bonds between endothelial cells
- Suppresses inflammation and vascular leakage
- Keeps blood vessels from a 'fragile' state to a 'healthy state'

■ Agonist
binds to a receptor, and
A substance that "activates" (activates)

What is ARDS?

■ What is ARDS (Acute Respiratory Distress Syndrome)?

It is a severe disease in which the lungs suddenly become inflamed, making it difficult to properly absorb oxygen. The fatality rate is high, about 30~40%, and management in intensive care units is required.

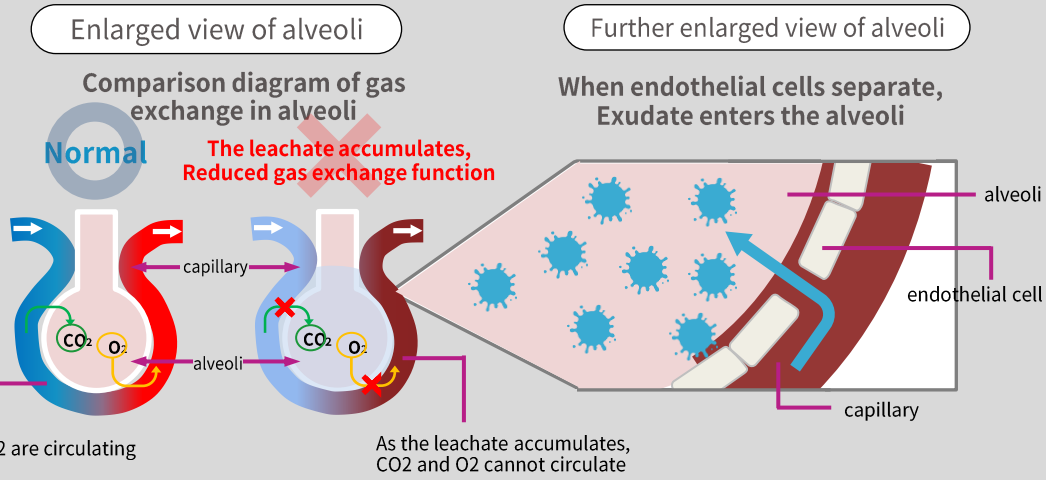
■ Pathology: "The state where the lungs become waterlogged"

Rapid inflammation of the lungs breaks the walls of alveoli and blood vessels, making it easier for blood components to leak out. As a result, 'pulmonary edema,' where exudate accumulates inside the alveoli, causing oxygen to stop reaching the bloodstream and causing severe shortness of breath.

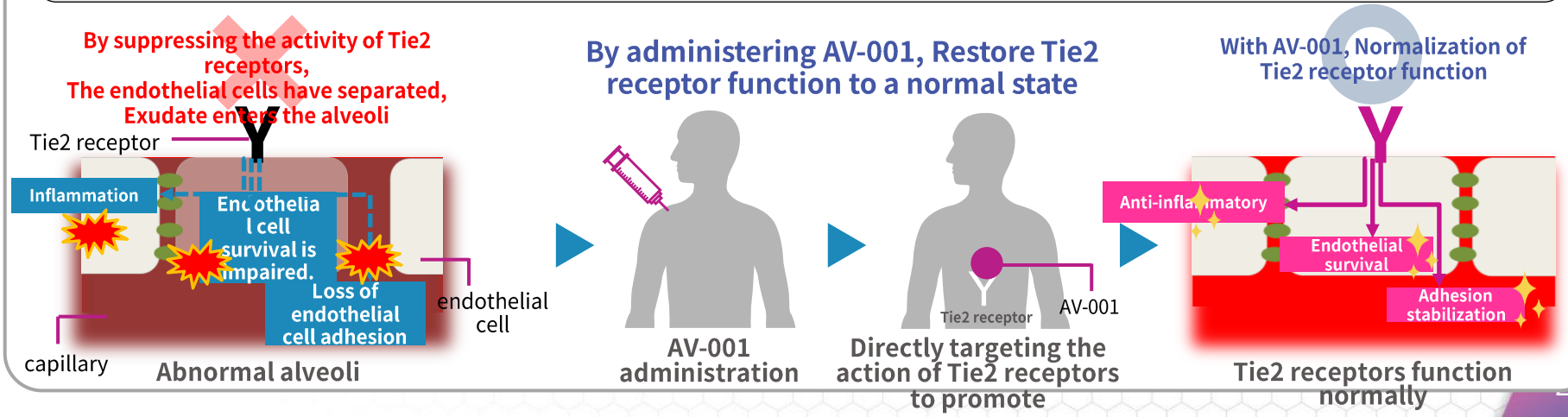
■ Current Standard Treatment (Symptomatic Therapy)

There are no effective specific treatments; management focuses on the following aspects.

- Breathing and posture management: Use of ventilators, lying face down, etc.
- Drug therapy: Suppression of inflammation with steroids
- Hydration regulation: Adjusting the amount of water in the body (fluid replenishment management)



Mechanism of AV-001 (Maintenance and Restoration of Vascular Physiological Function)



Tie2 Receptor Agonist (AV-001)

As a treatment for acute respiratory distress syndrome (ARDS)
Preliminary Phase II clinical trial conducted in the United States

Registration of the planned number of cases has been completed, and additional registrations for those who have been dropped are currently being carried out.

Registration to be completed in Q3/ 2026

Designated as Fast Track by the FDA

- More frequent meetings and communications with the FDA
- Expedited approval and priority screening
- You can submit application documents step-by-step and undergo review

Future Developments of AV-001

Started a new physician-initiated trial

Suppressing cognitive decline by reducing cerebral edema associated with hemodialysis and to evaluate the effect of maintaining white matter function

Up to 90% of patients with end-stage renal failure use hemodialysis.

It can cause symptoms such as confusion, delirium, and long-term cognitive decline
In particular, among patients aged 55 and older, 70% develop moderate to severe cognitive impairment

Received a grant from the Canadian Heart and Stroke Foundation
Aiming to stabilize cerebral blood vessels that experience significant circulatory stress during dialysis
Starting administration to patients in March 2026

Signed a contract to expand AV-001's target diseases to all diseases

Zokinvy

About Zokinvy

Hutchinson-Gilford-Progeria syndrome (HGPS) and Treatment for progeroid laminopathy (PDPL) with processing imperfection

Target Diseases

The so-called 'HGPS' and 'PDPL' are known as premature aging in infants

Precocious aging is a general term for diseases in which signs of aging appear earlier than one's actual age and throughout the body

Because symptoms of aging progressing faster than one's actual age, it is called "premature aging."

The average life expectancy of patients with HGPS is approximately 14.5 years.

Effectiveness

Hutchinson-Guilford-Progeria Syndrome (HGPS)
In patients, mortality is reduced by 72%,
Data shows that the average survival period can be extended by about four years

Safety Profile

Many HGPS patients have continued Zokinvi treatment for over 10 years,

Reported side effects include vomiting, diarrhea, and nausea, most of which are mild or moderate

About Zokinvy

Sales start on May 27, 2024

- 2022 May: Exclusive sales agreement with Eiger for 'Zokinvi' in Japan Signed an exclusive sales agreement with Eiger
- 2022 May: Orphan drug Designation Application
- 2023 March: Orphan drug be designated
- 2023 May: Applied for manufacturing and marketing approval for 'Zokinvi' in Japan
- 2024 January: Acquisition of Manufacturing and Sales
- 2024 May: **Sales launched in Japan**

Zokinvy (Ronfarnib)



[Drug Price]

Left: 91,796.40 yen / 50mg (1 capsule)

Right: 136,544.00 yen / 75mg (1 capsule)

[Indications, Effects, or Performance]

Hutchinson-Guilford-Progeria syndrome and Progeroid laminopathy with processing impairment

<Usage and Dosage or Usage>

Starting dose as lonafarnib at 115 mg/m^2 (Body surface area) is administered orally twice daily during or immediately after meals, and after 4 months, the maintenance dose of 150 mg/m^2 (body surface area) is administered orally twice daily during or immediately after meals.
Dose reduction should be made as appropriate according to the patient's condition.

AnGes Clinical Research Laboratory (ACRL)

What is Anges Clinical Research Laboratory? (ACRL)

A clinical laboratory specializing in rare genetic disease testing primarily focused on rare genetic disease testing

Anges Clinical Research Laboratory

Entrusted with expanded newborn screening testing services



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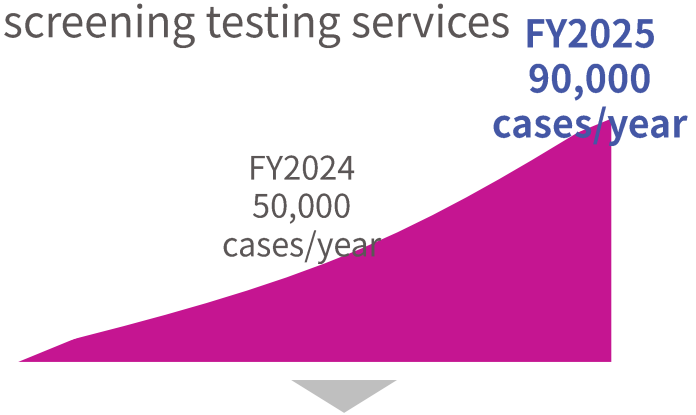


Going forward, we plan to expand our contracted clients. Expanding the range of diseases subject to testing

Testing for newborns

Newborn mass screening (public mass screening)

- Free of charge for newborns born in Japan
- Congenital metabolic disorders (such as phenylketonuria) and Endocrine disorders (such as congenital hypothyroidism)

Expanded Newborn Screening

- **Paid service for those who wish**
- **Diseases not subject to mass screening**
  (SMA, SCID, Pompe disease, mucopolysaccharidosis, etc.)

About Rare Genetic Disease Testing

For rare hereditary disease testing,
There are "screening tests," "genetic tests," and "biomarker tests."

Rare genetic disease testing

Screening Tests

Testing for possible hereditary diseases in newborns

Detailed Examination and Genetic Testing
(Definitive diagnosis)

Screening tests suggest possible disease
If diagnosed, the presence or absence of a disease is confirmed.

Biomarker Testing
(Monitoring Treatment Effectiveness)

After starting treatment, we will assess the effectiveness and improvement status
Providing data for decision-making

Newborn mass screening

- **Implemented at public expense for brand-new newborns born in Japan**
- **Congenital metabolic disorders** (phenylketonuria) **and Endocrine disorders** (such as congenital hypothyroidism)

Expanded Newborn Screening

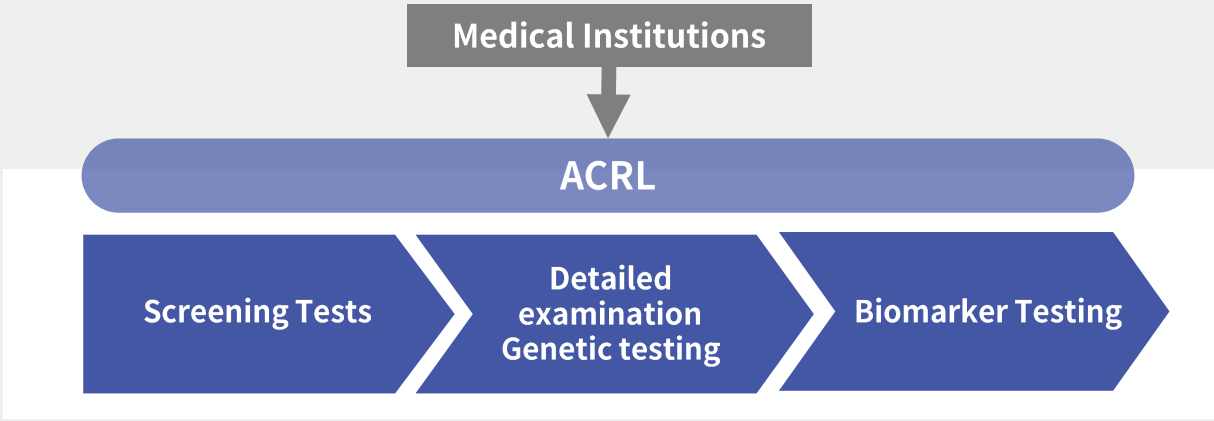
- **Paid service for those who wish**
- **Diseases not subject to mass screening** (Pompe disease, mucopolysaccharidosis, etc.)

One-stop rare genetic disease testing

In lysosomal diseases, false positives* are frequently detected during screening.
Heavy burden on families and healthcare workers

Biomarker testing to screen for false positives
Developed and began contracting testing methods for secondary screening

At ACRL, we conduct various tests related to rare genetic diseases.
Building a system that enables consistent delivery

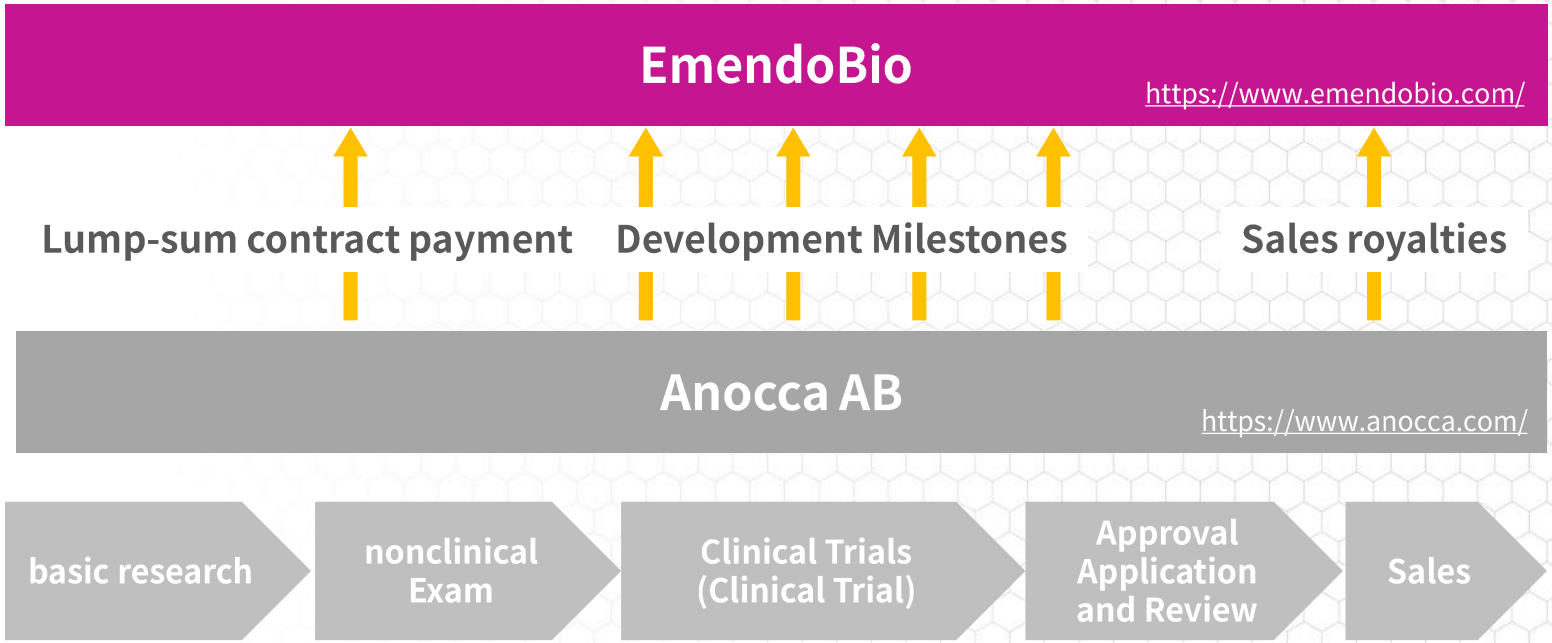


*What is a false positive... Screening test results that are positive even though there is no illness

EmendoBio

EmendoBio License Agreement Details

March 14, 2024
EmendoBio Licenses with Sweden's Anocca
(EmendoBio grants exclusive rights to use OMNI nuclease for genome editing)

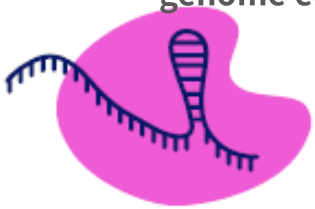


TCR-T Cell Therapy

Anocca uses OMNI nuclease, developed by EmendoBio,
Advancing development of TCR-T cell therapies targeting KRAS protein mutations in solid tumors

EmendoBio

More accurate than the conventional Cas9, OMNI Nuclease enables efficient genome editing

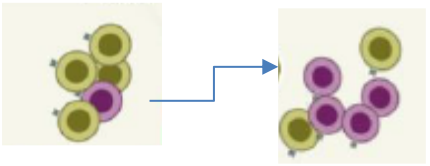


emendobio

+

Anocca AB

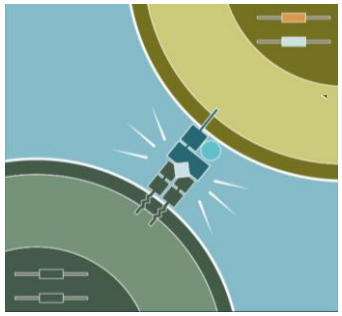
TCR that recognizes specific antigens
Selected T cells to express them,
Cultivation Techniques



anocca

Recognizing cancer cell-specific antigens Fabrication of artificial T cells (TCR-T cells)

Anocca possesses a library of TCR receptors specific to various cancers.
Using EmendoBio's OMNI nuclease,
TCR-T cells are produced by incorporating the target cancer's TCR receptor gene



About Immunotherapy

We collect T cells, which are one of the immune cells the patient possesses,
A treatment method that strengthens the fight against cancer and restores it to the body

Because the patient's own immune cells are the foundation,
No risk of legitimate organizations being attacked

Blood cancer
Caused by abnormalities in hematopoietic tissue
Increase without forming a clump
(Leukemia, malignant lymphoma, myeloma, etc.)

CAR-T Cell Therapy

CAR-T cell therapy involves introducing chimeric antigen receptors into T cells using genetic modification technology, allowing them to proliferate outside the body and then injecting them into patients. CAR-T has achieved great success in hematologic cancers, but since it can only recognize antigens on the cell surface, there are challenges in applying it to solid tumors.

solid cancer
Formed by the gathering of cancer cells
Increase in clusters
(Pancreatic cancer, lung cancer, colon cancer, esophageal cancer, etc.)

Anocca AB
EmendoBio
TCR-T Cell Therapy

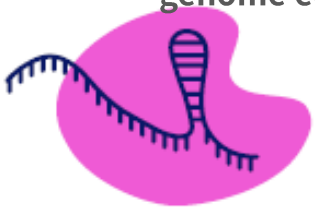
Artificial T cells (TCR-T cells) expressing TCR receptors, which recognize cancer-related antigens within cells, are applicable to solid tumors, unlike CAR-T targeting hematologic tumors such as CD19 CAR-T. They are attracting significant attention as a treatment with minimal side effects for inoperable, refractory solid tumors.

Joint research with Stanford University

Using OMNI nuclease developed by EmendoBio,
Started joint research and development of novel cancer treatments with Stanford University

EmendoBio

More accurate than the conventional Cas9, OMNI Nuclease enables efficient genome editing



emendobio

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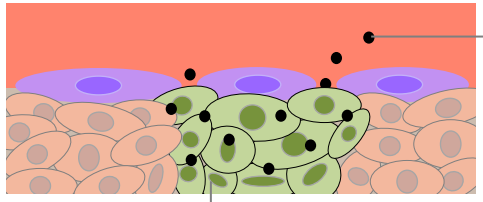
Stanford University

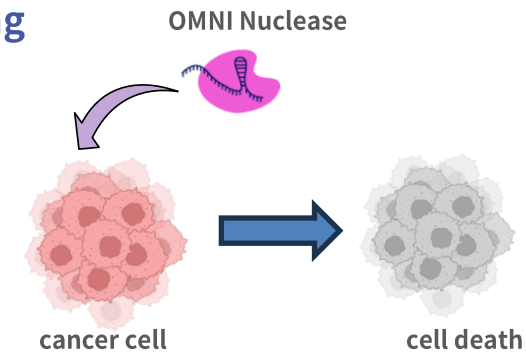
Drugs tailored to cancer tissue, Sending technology



Stanford University

Development of novel cancer genome editing therapies targeting breast cancer

At Stanford University, we have been conducting advanced research in the development of new cancer therapies, including tumor tissue-specific drug delivery technologies, cancer radiation therapy, and cancer immunotherapy. We develop therapies that kill only cancer cells by using EmendoBio's OMNI nuclease to perform genome-specific editing and reduce cancer cell resistance to treatment.



Genetic Innovation for All

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