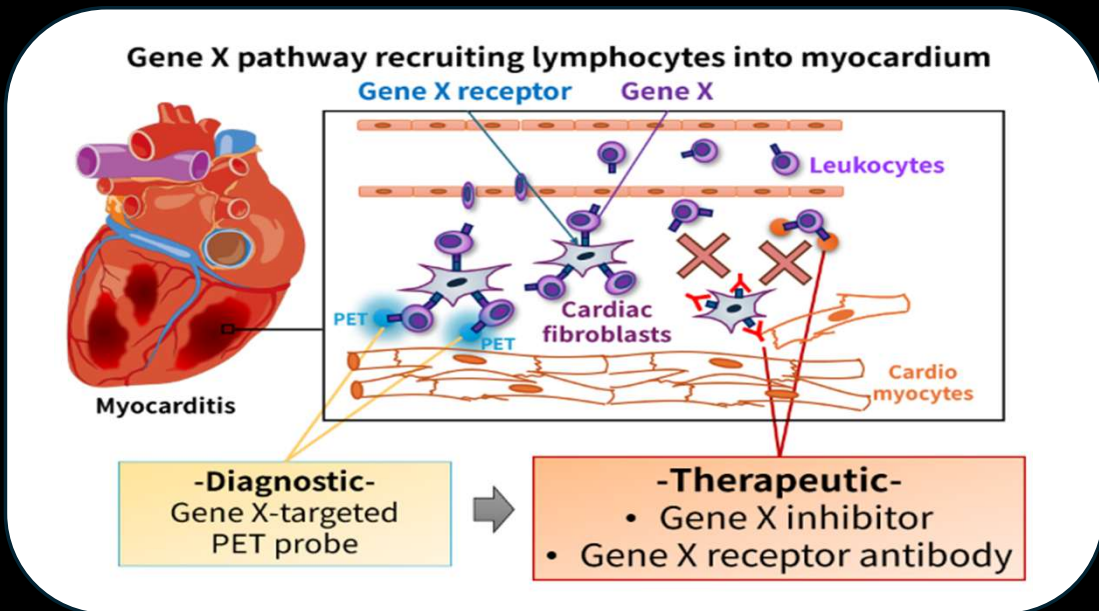


リンパ球性心筋炎の 予後改善が期待できる 遺伝子Xを標的とする 治療薬と診断法の開発



First-in-Class Gene X-Targeted Therapy for Myocarditis Enabled by a Proprietary PET Imaging Platform

独自のPETイメージングプラットフォームを活用した
心筋炎に対するファースト・イン・クラス
「遺伝子X」標的療法

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Executive Summary

Purpose

Develop a Gene X-targeted precision medicine strategy for lymphocytic myocarditis combining PET imaging with targeted therapy.

Current Status

- Gene X-targeted PET: clinical validation study ongoing.
- Gene X inhibitor: confirmed strong preclinical POC; under planning Phase II
- Gene X receptor inhibitor: next-generation antibody discovery program.

Mechanism / Modality

- Gene X/Gene X receptor signaling mediates lymphocyte recruitment into inflamed myocardium.
- Diagnostic: Gene X-targeted PET probe
- Therapeutic: small-molecule Gene X inhibitor; future Gene X receptor-targeted biologic.

Target Indications

Lymphocytic myocarditis, including ICI-associated and severe/fulminant myocarditis.

Competitive Advantages

- Targets a disease-driving trafficking mechanism rather than broad immunosuppression.
- PET enables patient stratification and treatment monitoring.
- Clinically de-risked therapeutic approach with biomarker-guided development.

Desired Collaboration

- Co-development/licensing of the Gene X inhibitor
- PET/companion diagnostic development
- Strategic partnership including next-generation Gene X receptor therapeutics

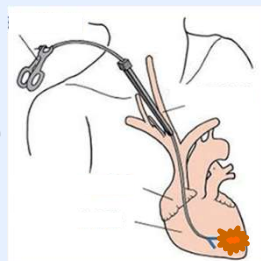
Backgrounds

Incidence of myocarditis is increasing

- ✓ Frequently affects **young adults** (20s)
- ✓ **1.8 million cases/year** worldwide
- ✓ **1.6-fold ↑** over 30 years

Diagnostic Challenges

- ✓ Endomyocardial biopsy
- ✓ **Invasive**
- ✓ High false-negative rate



Therapeutic Challenges

- ✓ **No established disease-specific therapy**
- ✓ **Mortality: 30–40%**
- ✓ No patient stratification for targeted therapy

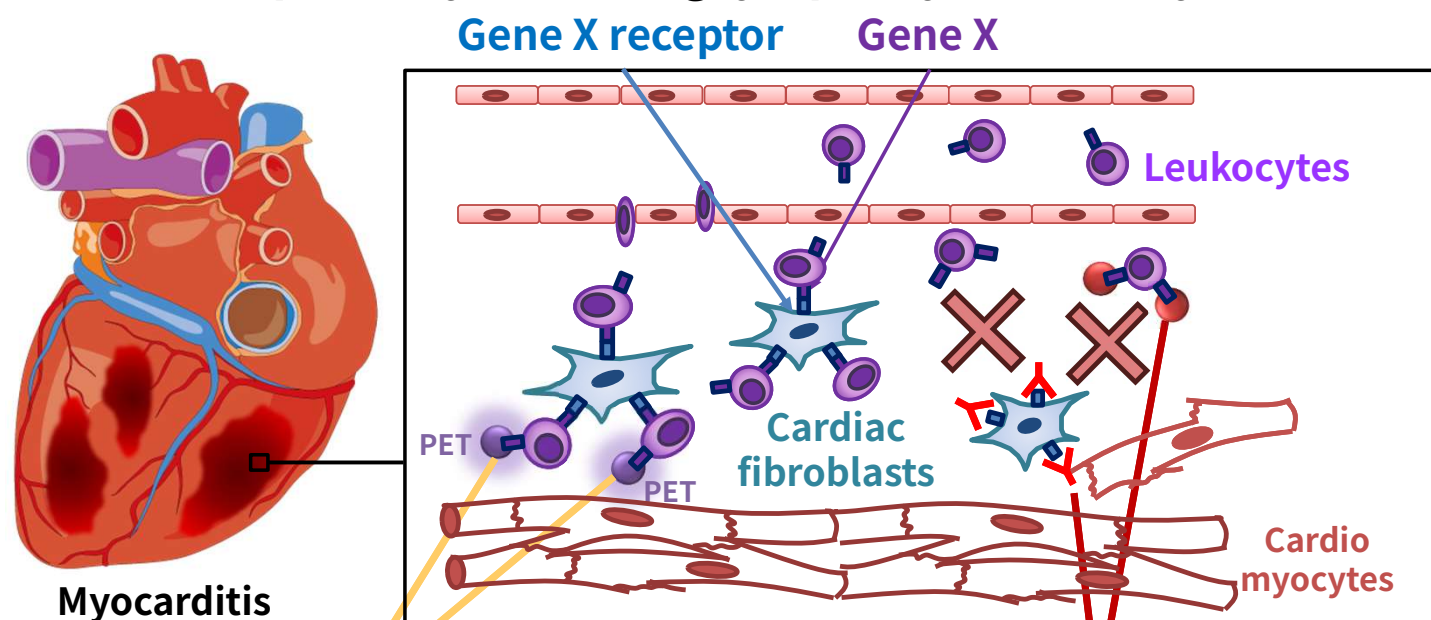
Targeted Goal; First-in-Class Gene X–Targeted Precision Therapy for Myocarditis

■ Two-Step Development Strategy

- **First Market Entry Program: Gene X Small Molecule Inhibitor**
 - ✓ Approved only in Japan for another inflammatory indication
 - ✓ Strong preclinical proof-of-concept in myocarditis
 - ✓ Gene X–targeted PET for clinical pathway validation
- **Market Extension Program: Gene X Antibody Development**
 - ✓ Novel antibody discovery underway
 - ✓ Lead optimization and future indication expansion

Key Data: A Biomarker-Enabled Precision Medicine Platform for Myocarditis

Gene X pathway recruiting lymphocytes into myocardium



-Diagnostic-
Gene X-targeted
PET probe



-Therapeutic-

- Gene X inhibitor
- Gene X receptor antibody

PET Imaging



**Gene X-Positive
Patients**



**Gene X Pathway
Inhibition**



Reduced Inflammation



Improved Outcomes

Other Data

Other available non-confidential data

- Preclinical POC data for the Gene X inhibitor
- Preclinical imaging data for the Gene X–targeted PET probe

After execution of Non-Disclosure Agreement

- Disclosure of target identity and detailed research data
- Detailed discussion of the development and collaboration strategy

Reference (Patents / Key Papers)

Patents:

- **Gene X-targeted PET probe:**
Filled in 2023
Country: JP, CN, US, EP
Applicants: The University of Osaka
- **Gene X inhibition for myocarditis:**
Filled in 2025
Country: JP, CN, EP
Applicants: The University of Osaka

Publications:

- Manuscripts on diagnostic and therapeutic proof-of-concept in preparation

Competitive Advantage

Gene X–Targeted Precision Medicine Combines
Clinical Readiness with Biomarker-Guided Patient Selection

Approach	Mechanism	Precision	Biomarker	Clinical Readiness
Steroids	Broad	Low	None	Available
Immunosuppressants	Broad	Low	None	Available
Gene X inhibitor	Targeted	High	PET	Phase II planning
Gene X receptor antibody	Targeted	Very High	PET	Discovery Stage

Gene X inhibition combines a de-risked therapeutic strategy with PET-enabled patient stratification, potentially improving both treatment efficacy and clinical trial success.

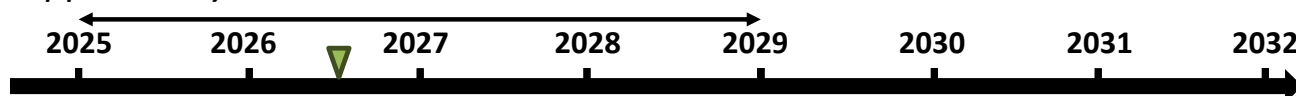
Research & Development Roadmap

Two-Step strategy

Step 1: de-risked small molecule approach (Gene X)

Step 2: more ideal novel antibody approach (Gene X receptor)

Supported by AMED Clinical Research 2025-2028



Step1. Repurpose Gene X inhibitor

Gene X-targeted
PET probe

Safety study
3 cases
normal adults

Clinical Validation Study
Myocarditis 20 cases
Non-myocarditis 30 cases

Value Inflection

1. Human PET validation
2. Patient stratification for future Gene X inhibitor trials

Gene X inhibitor

Regulatory
Preparation

**Investigator-initiated
Clinical trial phase2**

Myocarditis 60 cases
Placebo vs Drug

Value Inflection

3. Clinical POC enabling pharma partnership

Step2. Development 1st-in-class Gene X receptor antibody

Gene X receptor
antibody

Antibody
discovery

Lead
candidate
selection

Value Inflection

4. Lead antibody candidate enabling Series A financing

Our Team

Experienced Team Bridging Science, Clinical Development, and Commercialization

Founder / Scientific Lead

Ken Matsuoka, MD, PhD

Associate Professor
Medical Biochemistry
The University of Osaka

CEO•CSO

- Experienced biotech CEO / CSO to be recruited
- CMC, clinical development, regulatory functions will be outsourced through competitive site selection

Clinical & Translational Team

Department of Cardiovascular Medicine

- Yasushi Sakata

Department of Radiology

- Tadashi Watabe

Medical Center for Translational Research

- Yoshiaki Yamazaki

Business

Global Marketing Office

- Yoshikazu Kambayashi
- Masaharu Mori

IP & Licensing

Intellectual Property Department

- Takeshi Yoshioka
- Tomoyuki Okubo

**Integrated expertise spanning science, clinical development, IP, and commercialization.
Actively recruiting an experienced biotech CEO and company-building team.**

Proposed Partnership Scheme;

1. Seeking:

- Seed financing: JPY 640M (~USD 4.0M)
- Co-founder / CEO
- Drug development expertise

2. Use of proceeds:

Program	Amount
Gene X inhibitor Phase II preparation	¥400M (\$2.5M)
Gene X inhibitor CMC / regulatory work	¥200M (\$1.25M)
Gene X receptor antibody discovery	¥40M (\$0.25M)

* More realistic numbers will be determined after study plan is finalized

3. Expected milestone:

- Clinical development package for Gene X inhibitor
- Lead Gene X receptor antibody candidate
- Pharma partnering opportunity