English translation for reference purposes only. In case of any discrepancy, the Japanese version shall prevail

The Third Quarter of 2026 Business and Financial report

MODALIS THERAPEUTICS CORPORATION
TSE 4883



November 12th, 2026

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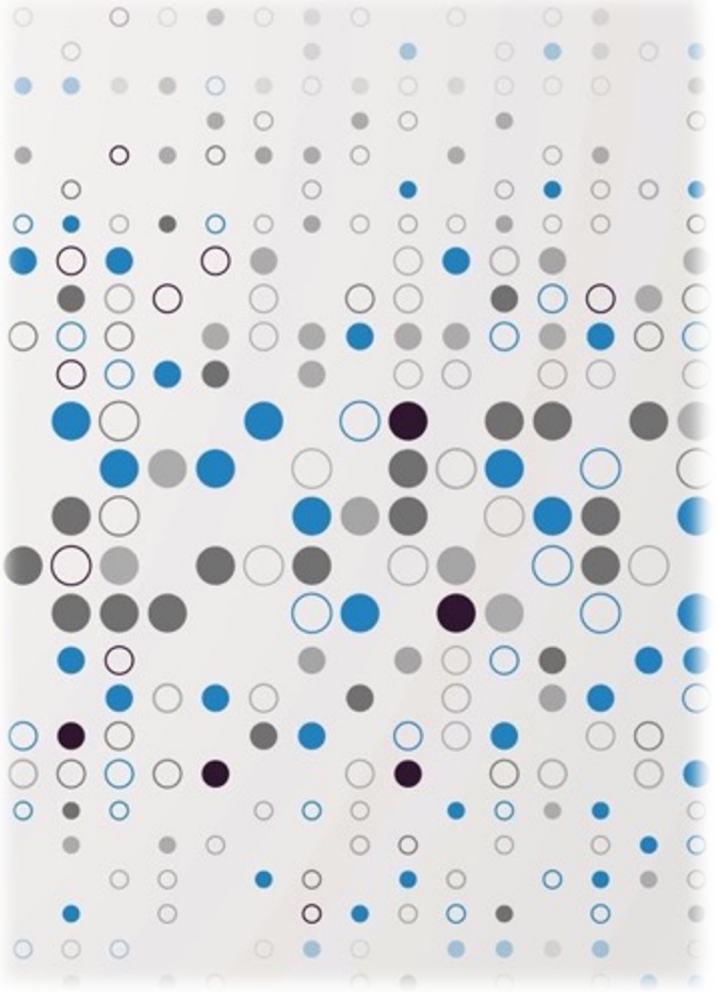
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As requested by TSE, This document is updated once a year, and disclosure is scheduled to be made around March of each year. In the event of significant changes in the business plan, changes may be made irregularly,

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0. Corporate Overview



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Corporate Information (As of September 30th, 2026)

MODALIS

Every Life Deserves Attention

Name	Modalis Therapeutics Corporation (Ticker symbol: 4883)
Foundation	Jan 2016
President CEO	Haru Morita
HQs	3-11-5 Nihonbashi-Honcho, Nihonbashi-Lifescience-Bldg.2 7F Chuo-ku, Tokyo 103-0023 Japan
US subsidiary	Modalis Therapeutics Inc. (43 Foundry Avenue, Waltham, Massachusetts)
Capital stock	1,159 million yen
Outstanding share	113,634,098 shares of common stock
Number of employee	17 (including 8 Ph.D.) (4 in Japan, 13 in US)

What is Modalis?

One Gene Regulation Platform
CRISPR-GNDM®
an epigenome editing technology
↓
Multiple Disease Applications



Turning beneficial genes **ON**
and harmful genes **OFF**

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MODALIS Value Highlights

Building the Next Generation of Genetic Medicines

Platform: CRISPR-GNDM®

Programmable gene activation and repression without DNA cutting

1st company advancing CRISPR-based gene activation therapeutics toward the clinic

4 Programs across neuromuscular diseases

Multiple therapeutic targets validated in vivo

IND MDL-101 is Making Progress Toward

Competitive Advantages

Gene Regulation Without DNA Cleavage

Demonstration of Long-Term Gene Expression Regulation

Applicable to neuromuscular to CNS disorders

Establishing tissue-selective AAV Delivery

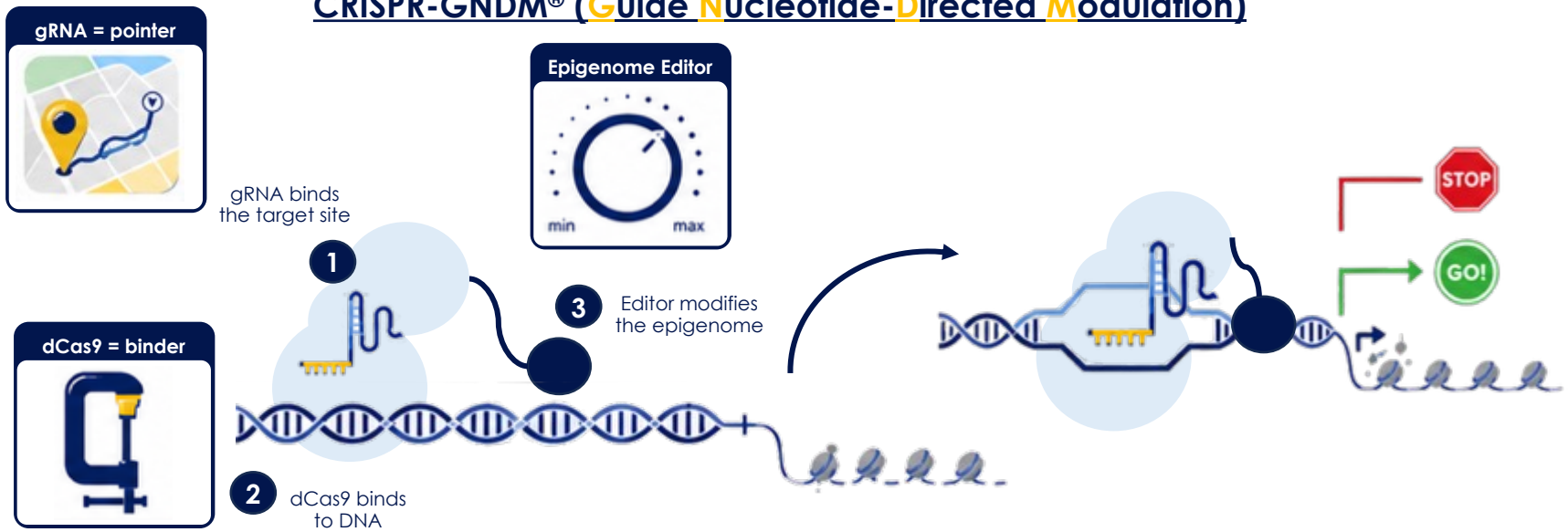
A robust intellectual property portfolio

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CRISPR-GNDM®'s mechanism of action

GNDM restores the controls target protein expression through epigenetic regulation

CRISPR-GNDM® (Guide Nucleotide-Directed Modulation)



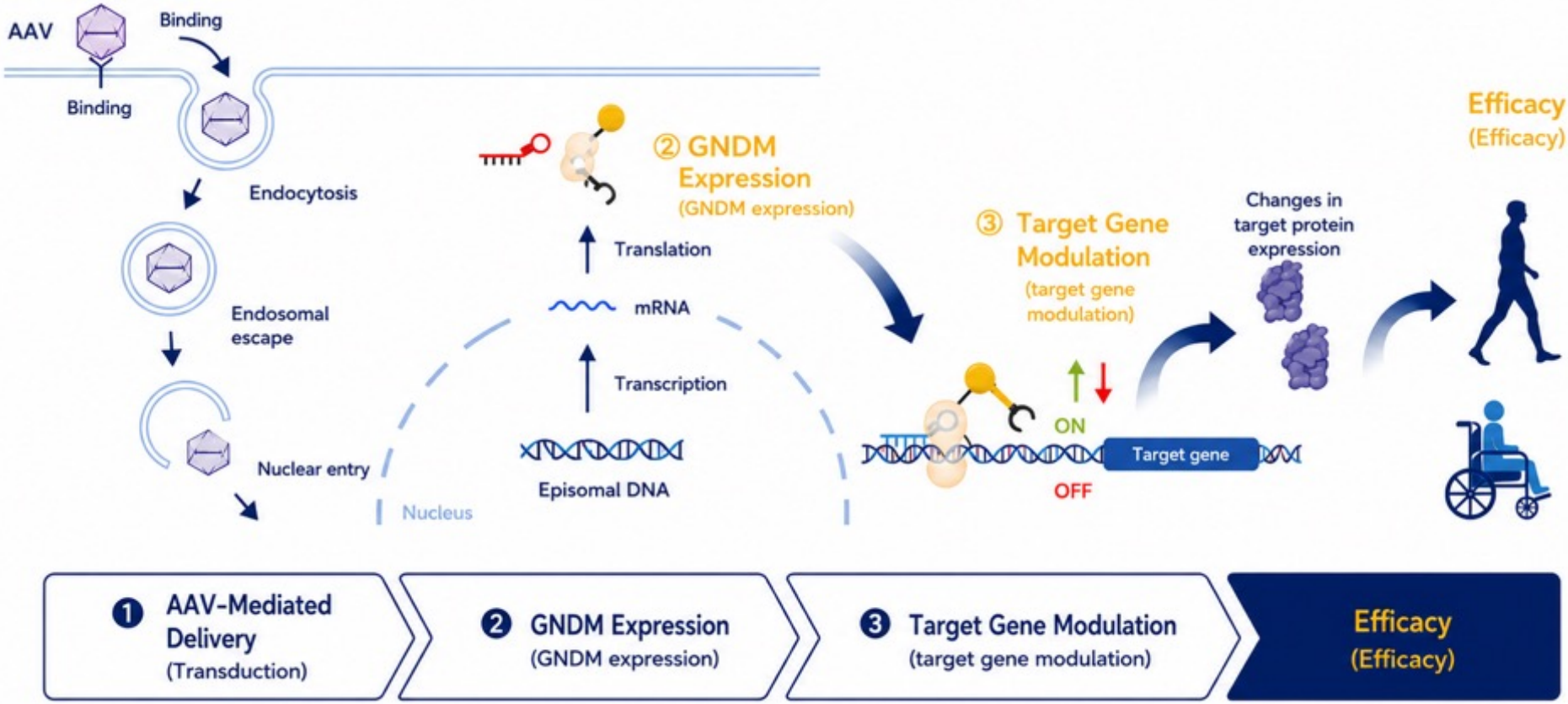
1 Guide RNA (gRNA) Directs the system to the target genomic site.	**2 dCas9** Binds to the DNA without cutting (no double-strand breaks)	**3 Epigenome Editor** Modifies the epigenome to precisely turn gene expression ON or OF

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Three Steps from Dosing to Therapeutic Efficacy

AAV delivers GNDM to target cells, GNDM is expressed as a protein, and then modulates the target gene to generate therapeutic benefit



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Updated

Our core competency = GNDM payload

intellectual property, target selection expertise, drug discovery engine, and the selection of capsids and our subsequent development capabilities

IP



A robust IP foundation for our proprietary GNDM technology

Selecting target



Extensive knowledge and experience in the identification and evaluation of optimal targets for gene therapy

Discovery Engine



A Drug Discovery Engine that Accelerates Design, Evaluation, and Optimization

Capsid Evaluation



Expertise that drives the process from selecting the optimal capsid and development

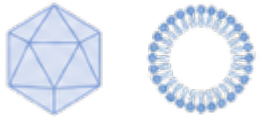
Payload
=CRISPR-GNDM®



Gene Therapy



Vector



In collaboration with

JCR Pharmaceuticals Co.,Ltd.
BROAD INSTITUTE
EXEGENESIS BIO
affinia THERAPEUTICS

By integrating these strengths, we will accelerate the realization of gene therapy and deliver value to patients.

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Our vision: *Every Life Deserves Attention*

Leveraging CRISPR Epigenome Editing technology to provide fundamental solutions to rare diseases

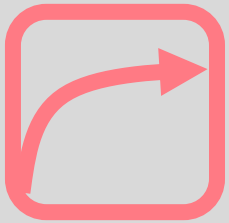
Key Benefits of Our CRISPR-GNDM® Technology



No DNA Cutting
Regulates gene expression without altering DNA



Single dose
Doesn't require Repeated dosing



Long-lasting
Sustained effect for years or decades



Disease Modifying
Aims to provide transformative benefit

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Approval Status of GTx

FDA approved 3 in 2026 including 2 of Ultragenyx's GTx that were approved in Aug and Sep.

In vivo gene therapies approved by US FDA

Trade Name	Year	Price	Indication	Manufacturer	Vector	Route	Patient Population / Comment
LUXTURNA	2017	$850,000 (both eyes)	Biallelic RPE65 mutation-associated retinal dystrophy	Spark/Roche	AAV2	SR	Ultra-rare inherited retinal disease; commonly estimated ~1,000–2,000 patients in the US
ZOLGENSMA	2019	$2.125M	SMA in patients <2 years with biallelic SMN1 mutations	Novartis	AAV9	IV	SMA incidence roughly 1 in 10,000 live births; US addressable population is a subset by age/diagnosis
HEMGENIX	2022	$3.5M	Adults with hemophilia B	CSL Behring	AAV5	IV	Hemophilia B is rare (~1 in 25,000–30,000 male births); eligible adult subset smaller
ROCTAVIAN	2023	$2.9M	Adults with severe hemophilia A	BioMarin	AAV5	IV	Hemophilia A is more common than hemophilia B, but gene-therapy-eligible subset is limited
ELEVIDYS		$3.2M	Duchenne muscular dystrophy	Sarepta	AAVrh74	IV	DMD occurs ~1 in 3,500–5,000 male births; current eligible population narrowed by ambulatory status and label limitations
BEQVEZ	2024	$3.5M	Adults with moderate-to-severe hemophilia B	Pfizer	AAVRh74 var	IV	Hemophilia B affects roughly 4 per 100,000 US males; eligible subset smaller
KEBILIDI		$3.95M	Adult and pediatric patients with AADC deficiency	PTC	AAV2	IP	Ultra-rare AADC deficiency; US treatable population is very small
ITVISMA	2025	$2,586,629.70	SMA in adult and pediatric patients ≥2 years with confirmed SMN1 mutation	Novartis	AAV9	IT	Broader prevalent SMA population ≥2 years; Reuters cited ~9,000 people with SMA in the US overall
OTARMENI	2026	Provided free in US	OTOF-related hearing loss	Regeneron	Dual AAV	IC	Ultra-rare OTOF-related hearing loss; highly selected molecularly defined population
GENGLYCOS		$2.7M	GSDIa patients ≥8 years	Ultragenyx	AAV8-based	IV	Estimated 1,500–2,500 patients with GSDIa in the US; age and anti-AAV eligibility further narrow addressable pool
FAYUVI		$3.95M	MPS IIIA		AAV9	IV	Among the approx. 3,000 to 5,000 patients with MPS IIIA, those with "preserved neurodevelopmental function"

SR: Subretinal IV: intravenous IP: Intraputaminal (stereotactic neurosurgical administration) IT: Intrathecal IC: Intracochlear

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Epigenome editing competitive landscape

The epigenome editing sector continues to attract strong investor interest

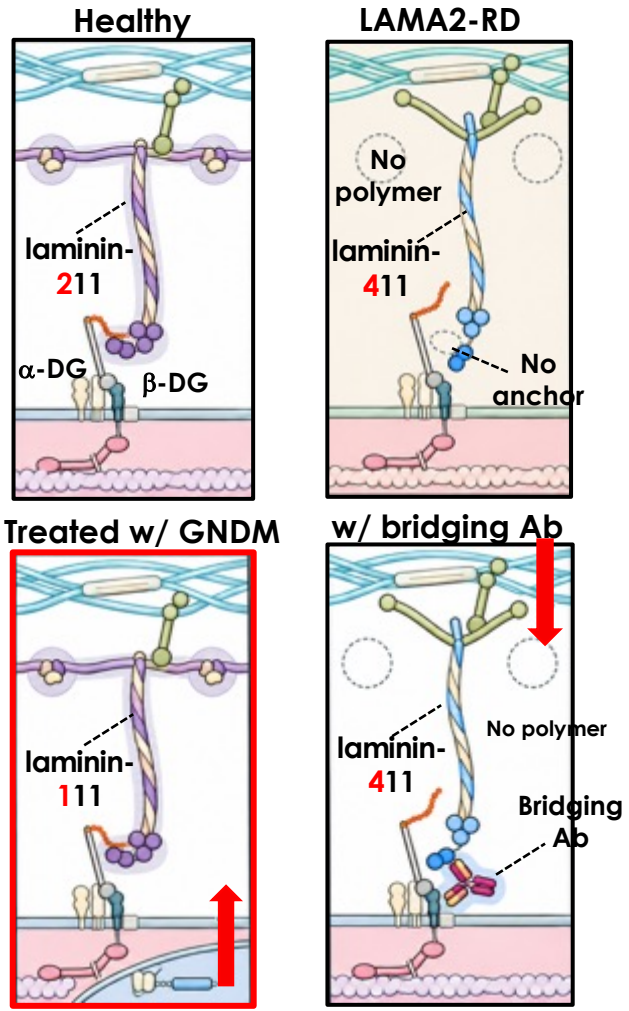
Company	Year Founded	Funding	Platform	Pipeline/Target indication	Stage of Development
MODALIS	2016	Public	CRISPR-GNDM x AAV	MDL-101 for LAMA2-CMD **Gene activation**	IND enabling
Tune	2020	Series B ($175M, 2025)	DNMT-KRAB fusion dCas9 x LNP	Une-401 for HBV Gene suppression	CTA approval from Hongkong, NZ and Moldova on HBV
nChroma	2021	Merged into nChroma (Dec 2024)	DNMT-KRAB fusion dCas9 x LNP	CRMA-1001 for PCSK9 Gene suppression	P1/2
Epicrispr	2022	**Series C ($90M Aug 2026)**	Cas12f-fused with demethylation enzyme x AAVrh74	EPI-321/FSHD Gene suppression	P1/2
Epigenic	2022	Series B ($60M, 2025)	dCas+editor x LNP	EPI-001 PCSK9 for hypercholesteremia Gene suppression	Chinese IIT
Scribe	2018	**IPO ($155.5M Jul 2026)**	dCas+repressor x LNP	STX1150 PCSK9 for hypercholesteremia Gene suppression	IND enabling
Mammoth	2017	Corp.Minority ($2024, $95M)	miniCas x editor x LNP	MB-111 APOC3 for Triglycemia Gene suppression	IND enabling

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Anthropic Enters LAMA2-RD Research

Announcement of October 27, 2026 | Fact Sheet

Item	Details
Announcement	Anthropic announced its acquisition of Mithril Medicine.
Acquisition	July 2026 (publicly announced Oct. 27, 2026)
Target diseases	LAMA2-related congenital muscular dystrophy (LAMA2-CMD) a-dystroglycanopathies
Mechanism of action	Bispecific antibodies designed to reinforce muscle fiber–basement membrane attachment
Early nonclinical results	Reported improvements in strength, muscle function and survival in LAMA2-CMD mouse models
Development stage	Early proof of principle in mice; candidate evaluation and optimization ongoing
Stated goals	Development candidate selection in 2027; potential clinical trials for LAMA2-CMD in late 2028 at the earliest



Note: Mouse data are preliminary; clinical safety and efficacy have not been established.
Source: Anthropic, "Early progress on accelerating drug development with Claude" (Oct. 27, 2026) anthropic.com/research/early-progress-drug-development

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1. Status of Business

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1. Business Highlights of the 3Q/2026

Highlights



01

MDL-101 Progress and Timeline



02

Progress in the other programs



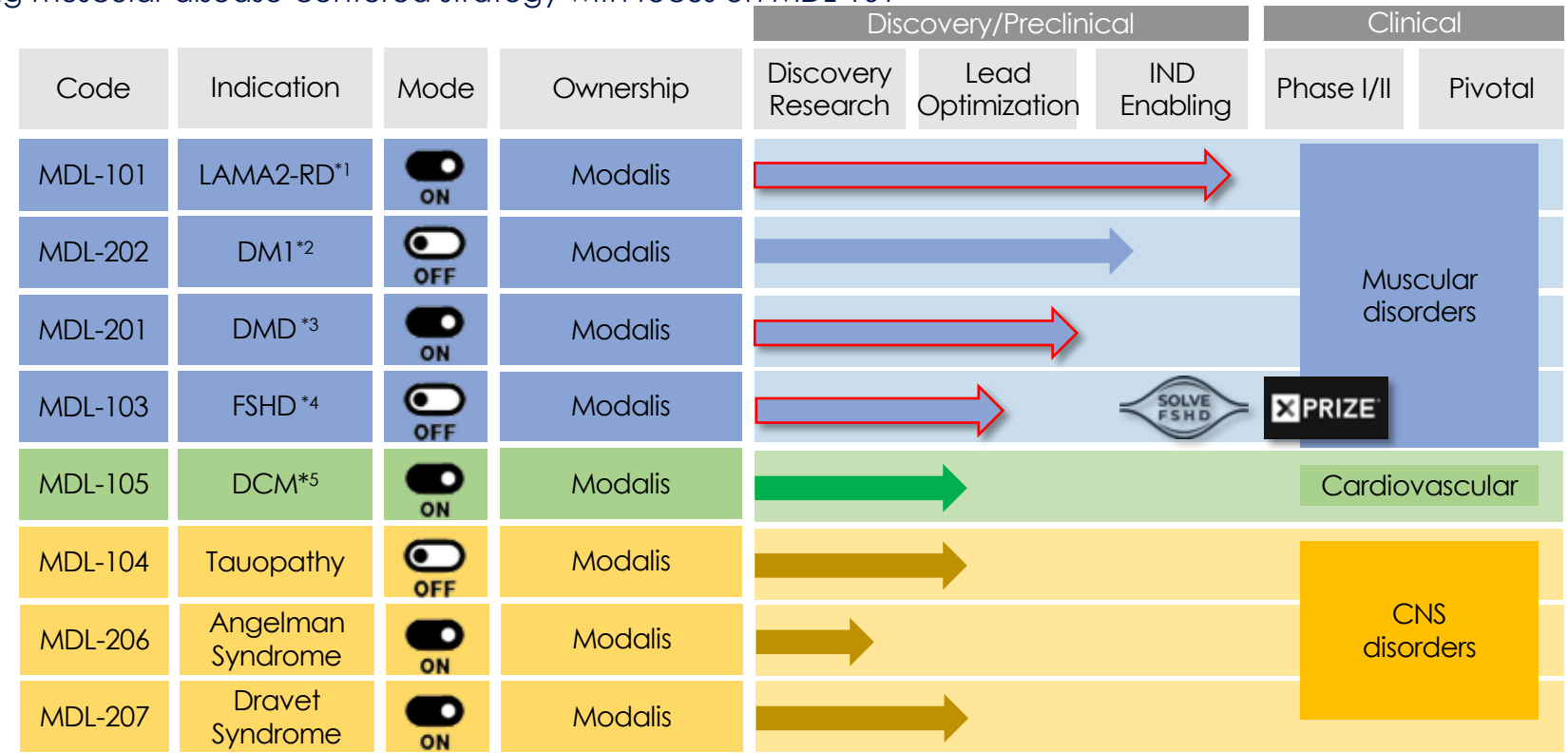
03

Finance and the other progress in the business

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The current pipeline of MODALIS

Taking muscular disease-centered strategy with focus on MDL-101



*1: LAMA2-related dystrophy
*2: Myotonic Dystrophy Type 1
*3: Duchene Muscular Dystrophy
*4: facioscapulohumeral muscular dystrophy
*5: Dilated Cardiomyopathy

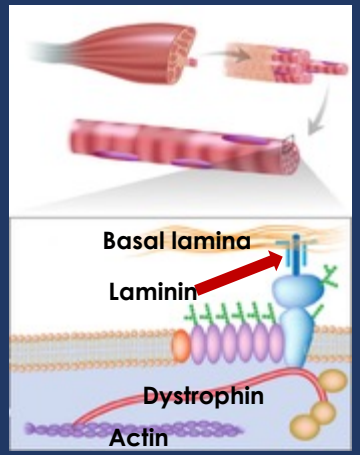
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LAMA2-RD (aka CMD1a)

Severe muscular dystrophy caused by loss of function mutation in LAMA2 gene

MDL-101

Potential to be the first LAMA2-CMD gene activation therapy



Prevalence	**8.3 in 1 million*** 2500 in US	
Disease Onset	Apparent at birth or within a few months after birth	
Disease Burden	Patients do not survive past adolescence	• Severe muscle weakness • Lack of muscle tone (hypotonia) • Little spontaneous movement • Joint deformities (contractures) • Heart problems and seizures
Disease Causing Gene	**LAMA2 mutation**	
Commercial opportunity	**$500M+**	



Source: *Estimating the Prevalence of LAMA2 Congenital Muscular Dystrophy using Population Genetic Databases (2023)

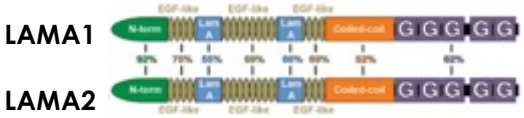
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Mechanism of Action of CRISPR-GNDM®

Restores muscle function by activating the sister gene LAMA1 in response to LAMA2 mutations

Protein structure of LAMA1 and 2

LAMA1 and LAMA2 are sister genes with high sequence homology (approximately 9 kb) that encode similar laminin a chains.

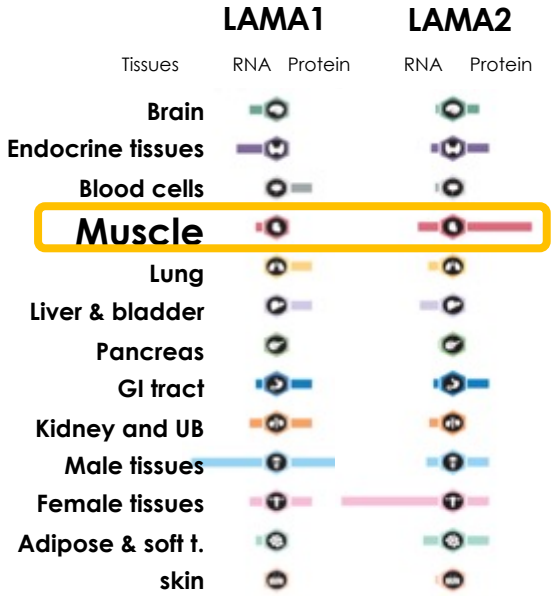


~9kb

(Too big to fit in single AAV)

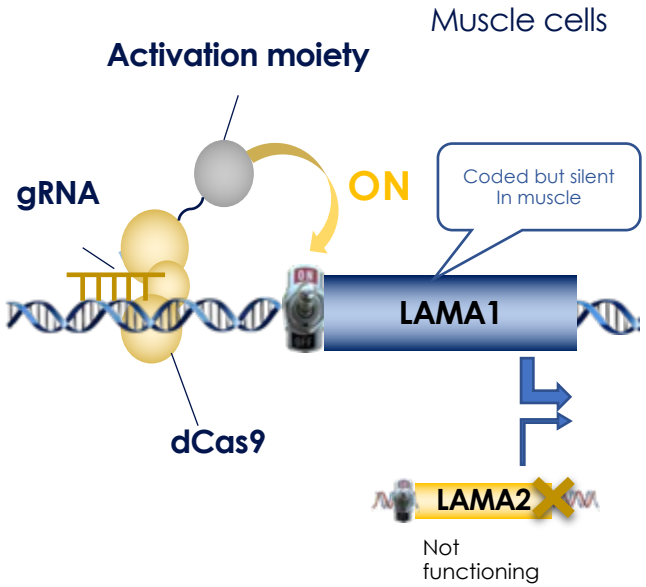
Due to their high homology, LAMA1 can compensate for the LAMA2

Expression pattern of LAMA1and 2 by tissues



LAMA1 is typically silent in muscle cells

Targeted LAMA1 activation using CRISPR-GNDM®



Enabling LAMA1 compensates for the loss of function caused by LAMA2 deficiency

★ CRISPR-GNDM® aims to restore muscle function in patients with LAMA2 deficiency by targeting the sister gene LAMA1

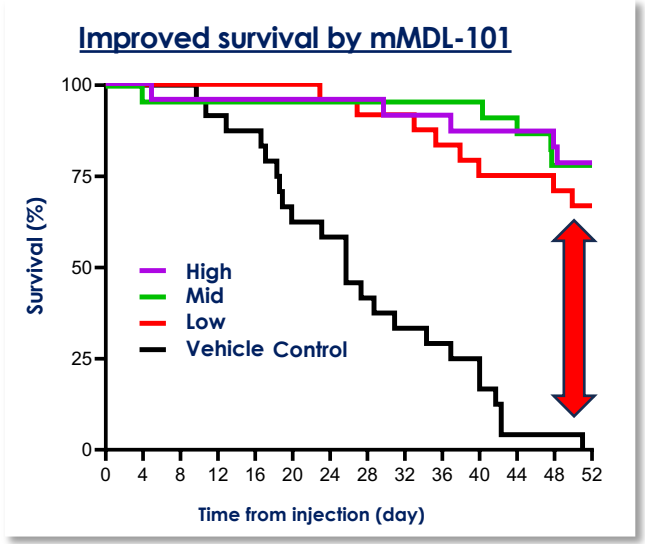
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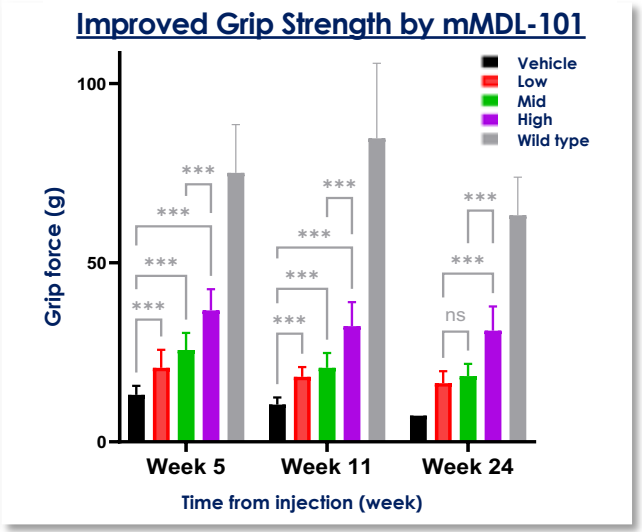
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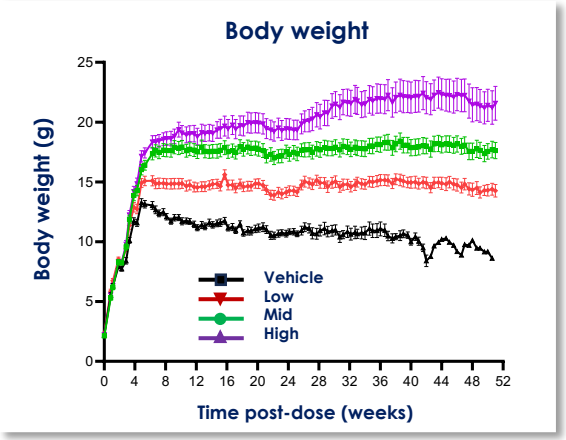
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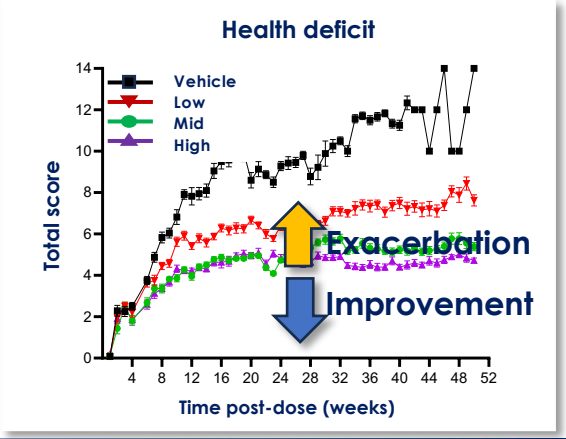
Dose dependent improvements in bw, grip strength, and survival in disease model

The significantly improved functionality and survival of the mice backed by sustained expression of GNDM and LAMA1









Sustained expression of GNDM and LAMA1

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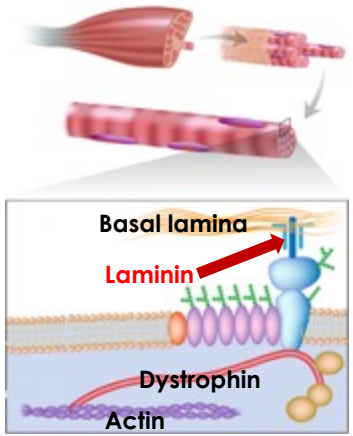
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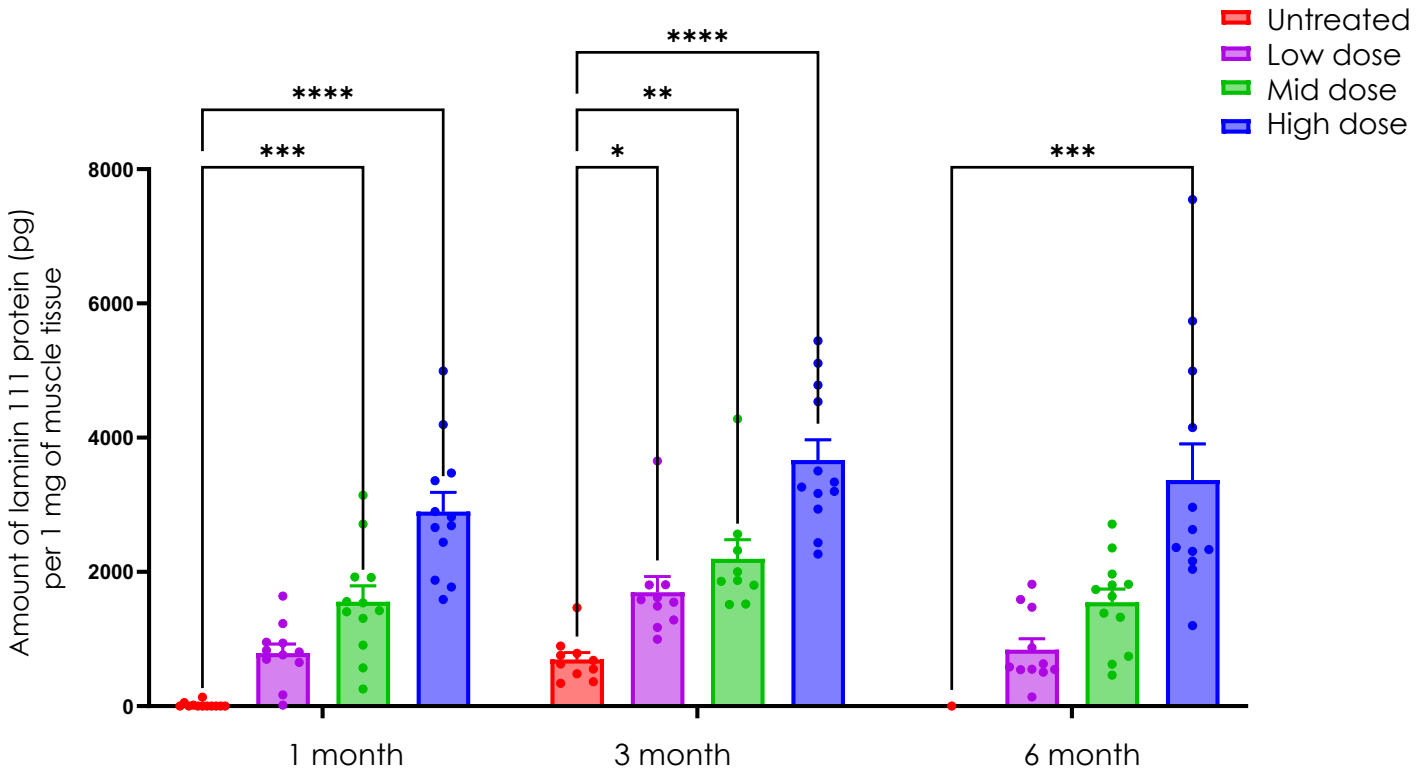
The increased expression and maintenance of LAMA1 underlying this effect

In disease model, laminin complex level increased in a dose-dependent manner and remained elevated even after 6 months after administration of MDL-101

New

Changes in the Amount of Laminin-111 in the Gastrocnemius Following Administration of mMDL-101





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Updated

MDL-101: Steady Progress on Key Milestones Toward IND Submission

Progress across GMP, GLP tox, and clinical trial readiness; IND submission targeted for late 2026–Q1 2027

1

CMC / MANUFACTURING

GMP Manufacturing Completed; Release Testing Underway

Manufacturing completed. Preliminary assessment confirms sufficient material for the planned clinical trial.
Product release expected in December 2026

2

GLP / NONCLINICAL

Additional GLP Toxicology Study Progressing Well

More than seven weeks post-dose, health conditions of animals including blood parameters have generally remained favorable
Three-month post-dose data expected in December 2026→incorporate into IND docs

3

CLINICAL / TRIAL READINESS

Clinical Site Shortlisted; Contract Discussions Advancing

Candidate clinical sites and primary investigator have been decided; contracting process for trial initiation underway.
Preparations for trial operations and regulatory activities continue toward IND submission

Advancing clinical readiness toward IND submission targeted for late 2026–Q1 2027

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Preparations for IND filing and dosing (Detailed)

Key tasks are progressing toward completion. The remaining steps are data analysis, data packaging, and the application process.

4 Key Factors for Initiating a Clinical Trial

1 rodent IND enabling study



- ✓ Study Design
- ✓ Development of Analytical Methods
- ✓ Dosing
- ✓ Data Readout
- ✓ Data Analysis

Verification of biomarkers and efficacy (survival, function, etc.)

2 GLP Tox study



- ✓ Study Design
- ✓ Development of Analytical Methods
- ✓ Dosing
- ☐ Data Readout(Dec)
- ☐ Data Analysis

Safety and Pharmaco-kinetic/dynamics Evaluation

3 GMP manufacturing



- ✓ Process development
- ✓ Analytical method development
- ✓ GMP manufacturing
- ☐ Release tests

Establishing a system to reliably supply clinical vectors

4 Clinical site preparation



- ✓ Selection of Clinical Trial Sites and PIs
- ☐ Protocol Coordination
- ☐ Logistics Confirmation

Recruitment and Alignment with Clinical Trial Sites

IND filing to FDA
Late 2026 ~ early 2027



Preparation of Data Package
→ Submission of IND Appl.
→ Clearance

FIH clinical trial
(1H 2027)



- Start of the FIH Trial in US
- Patient Recruitment
- FPFD*

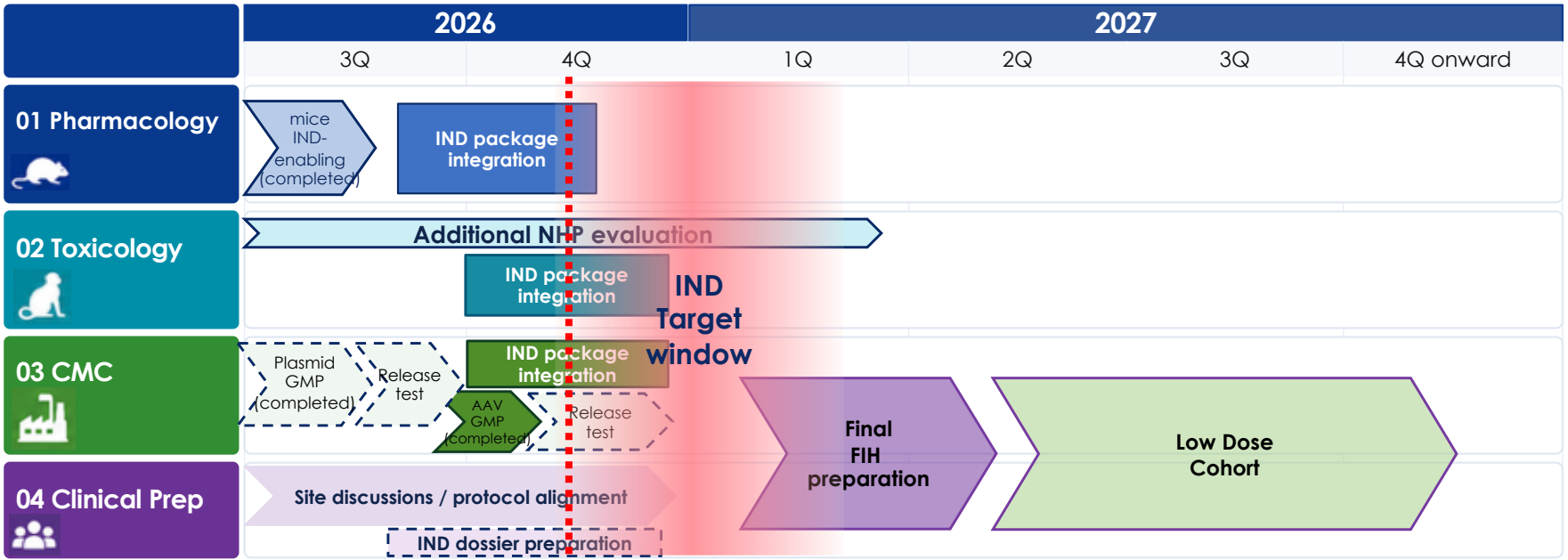
★ Steadily achieving key milestones toward clinical trial and aim to enter clinical trials asap

FPFD: First patient first dose(最初の患者さんへの最初の投与)
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MDL-101: Key Upcoming Milestones and Outlook for IND

As nonclinical, CMC and clinical activities advance, the target IND of late 2026 to 1Q 2027 become more realistic



Objectives

- IND process: Potential late-2026 initiation via expedited pathway; Q1 2027 submission target
- FIH: Begin promptly after IND clearance

Key assumptions & risks

- GLP tox: 3-month data in December; final report expected in Q1 2027
- AAV GMP complete; release testing underway
- Regulatory and site-contracting milestones remain under active management

MDL-101 aims to advance the IND process from late 2026 through Q1 2027 and move promptly toward FIH dosing.

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MDL-101: Regulatory Path Toward IND Submission

Advancing clinical readiness through FDA engagement, rare disease designations, and European regulatory preparations

US / FDA | Ongoing FDA interactions and regulatory designations support IND submission and clinical transition

1 FDA INTERACT
Talk
Initial regulatory interaction completed
Completed

2 Pre-IND Meeting
Docs
Development plan and study design discussed
Completed

3 Rare Pediatric Disease Designation
FDA
FDA designation obtained
Granted

4 Orphan Drug Designation
FDA
FDA designation obtained
Granted

5 IND Submission / FIH Preparation
IND
Preparing for IND submission and first-in-human dosing
In Progress

Europe | Preparing an orphan designation application to support global development

EMA **EMA Orphan Drug Designation** | Preparing orphan designation application | In Progress

Building on FDA interactions and regulatory designations, MDL-101 is advancing IND preparation and its transition toward clinical development.

Illustrative overview of key regulatory activities; does not indicate the timing of individual procedures.

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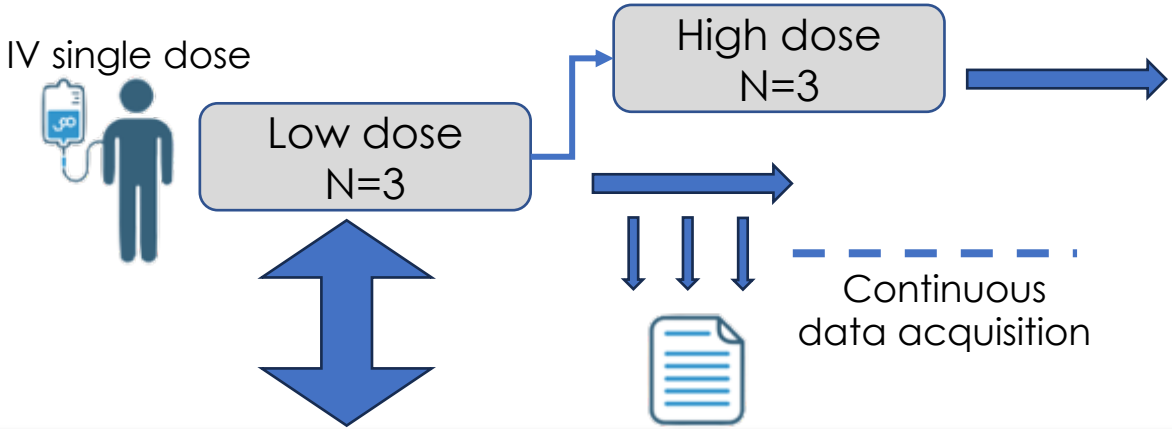
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MDL-101-001 Trial design

Open-label trial with two doses. Efficacy evaluated in comparison with natural history observation trial.

- Patients ≤ 5 years.
- Genetically confirmed, biallelic pathogenic *LAMA2* mutations.
- Severe LAMA2-RD clinical phenotype. Difficulty with independent walking or sitting.
- AAV9 TAb negative.

Phase1/2 Open-label dose escalating trial

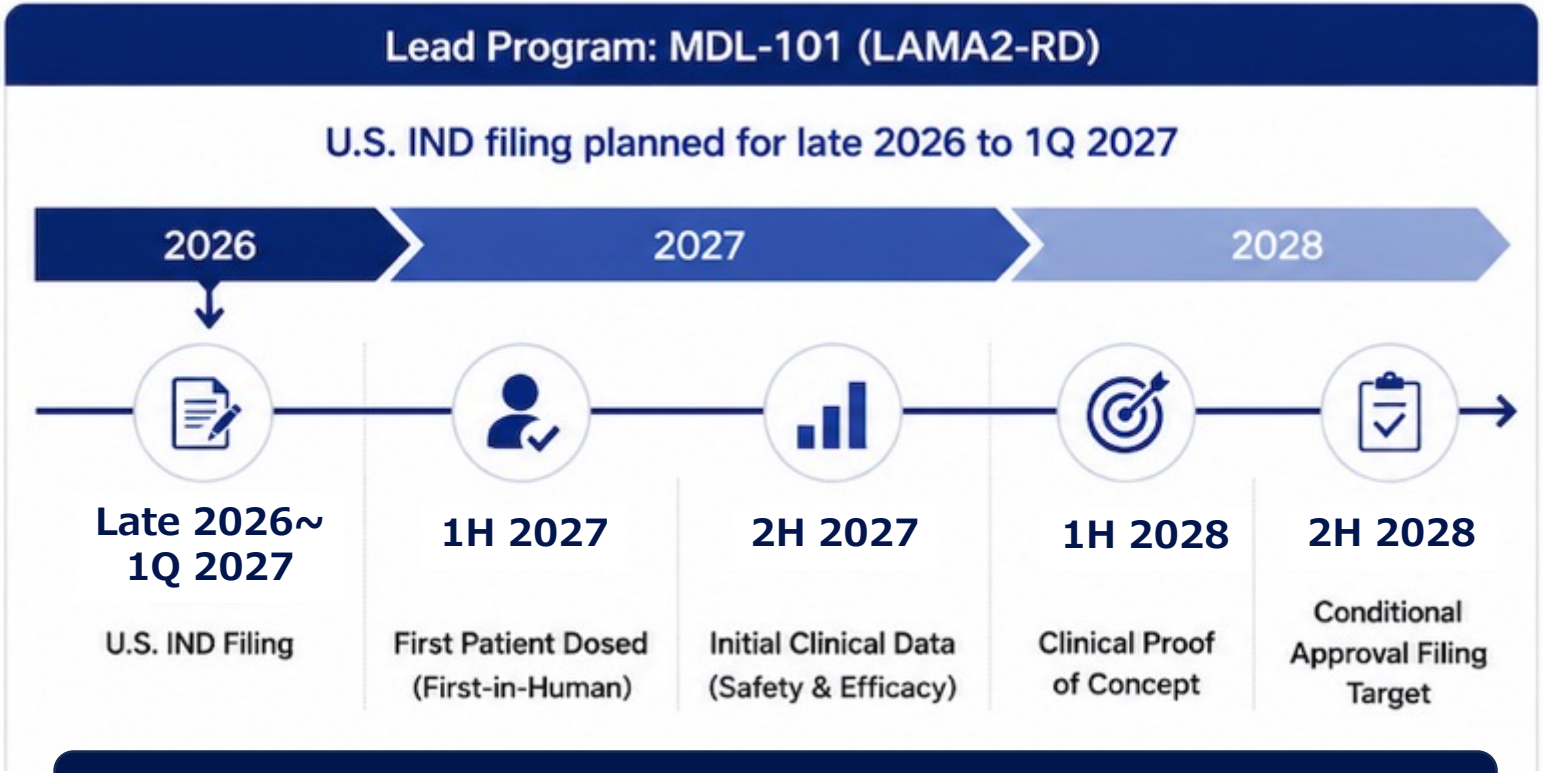


Retrospective	Natural History
Hinkley *et al.*, 2024 *(NCT04299321)* • 60 LAMA2-RD < 5 years old	**NCT06132750** (LAST STRONG) • 40 (est.) LAMA2-RD or SELENON-RM patients • Est. completion Sept 2026
Zambon et al., 2020 • 42 LAMA2-RD < 18 years old • 8 'complete deficient' < 5 years old	**NCT06354790** (Institut de Myologie, France) • 60 (est.) LAMA2-RD 2 to 15 years old • Est. completion Jan 2028
	NCT06503367 (READY CMD LAMA2) • 44 (est.) LAMA2-RD < 5 years old • Est. completion Sept 2028

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MDL-101 Development Priorities and Key Milestones

Development is proceeding as planned toward the IND, and the data is looking good.



The progress to date and the time-series data support the likelihood of reaching the IND application stage and are generally proceeding according to the planned timeline.

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Other muscle pipeline summary

MDL-101 remains the top priority as our pipeline advances and the GNDM platform expands.

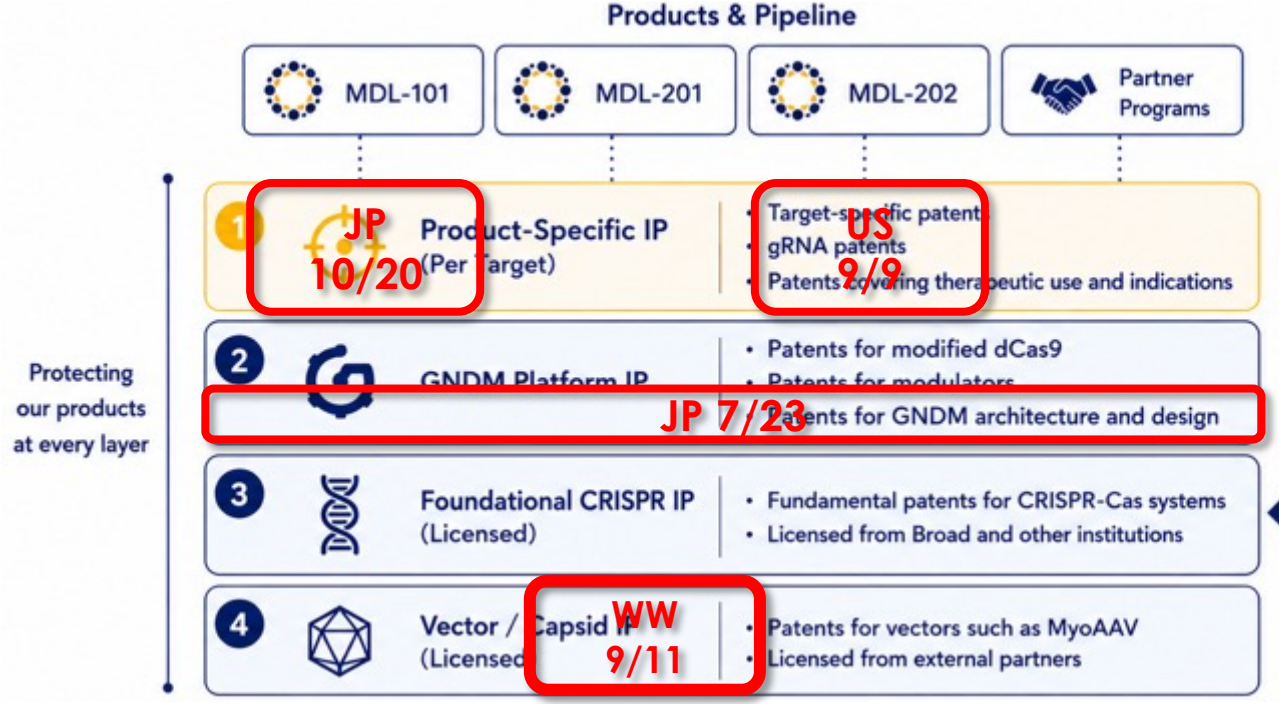
PROGRAM / MoA	INDICATION	STAGE	RECENT MILESTONES	NEXT STEPS
MDL-201 Utrophin activation	Duchenne muscular dystrophy (DMD)	Preclinical PoC optimization	• **Licensed** a muscle-targeted capsid through strategic collaboration with **Exegenesis Bio** • **US ODD application**	• Conduct a pilot NHP study to confirm target engagement
MDL-202 DMPK suppression	Myotonic dystrophy type 1 (DM1)	Preclinical PoC optimization	• Secured access to muscle-targeted capsid technology through **Affinia** collaboration • Potent DMPK suppression in mouse tissues, including cardiac muscle and diaphragm	• Expand preclinical dataset • Refine development strategy and explore partnering
MDL-103 DUX4 suppression	Facioscapulohumeral muscular dystrophy (FSHD)	Preclinical PoC optimization	• Demonstrated DUX4 suppression in mouse models • Advancing **Solve FSHD**-supported work and **UMASS** collaboration	• Expand preclinical dataset • Refine development strategy and explore partnering • Continue clinical-candidate development
Future Programs	CNS disorders, incl. Alzheimer's Angelman; titin cardiomyopathy (DCM)	Exploratory Research collaboration	• Advancing GNDM / JUST-AAV® integration with **JCR Pharma** • Early evaluation of multiple CNS indications	• Pursue preclinical proof of concept • Explore partnering opportunities

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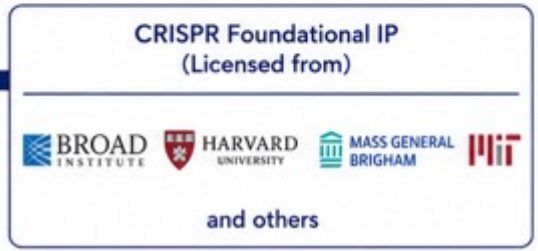
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Significant progress on IP

Three patents have been granted since the end of the second quarter and capsid license for MDL-201



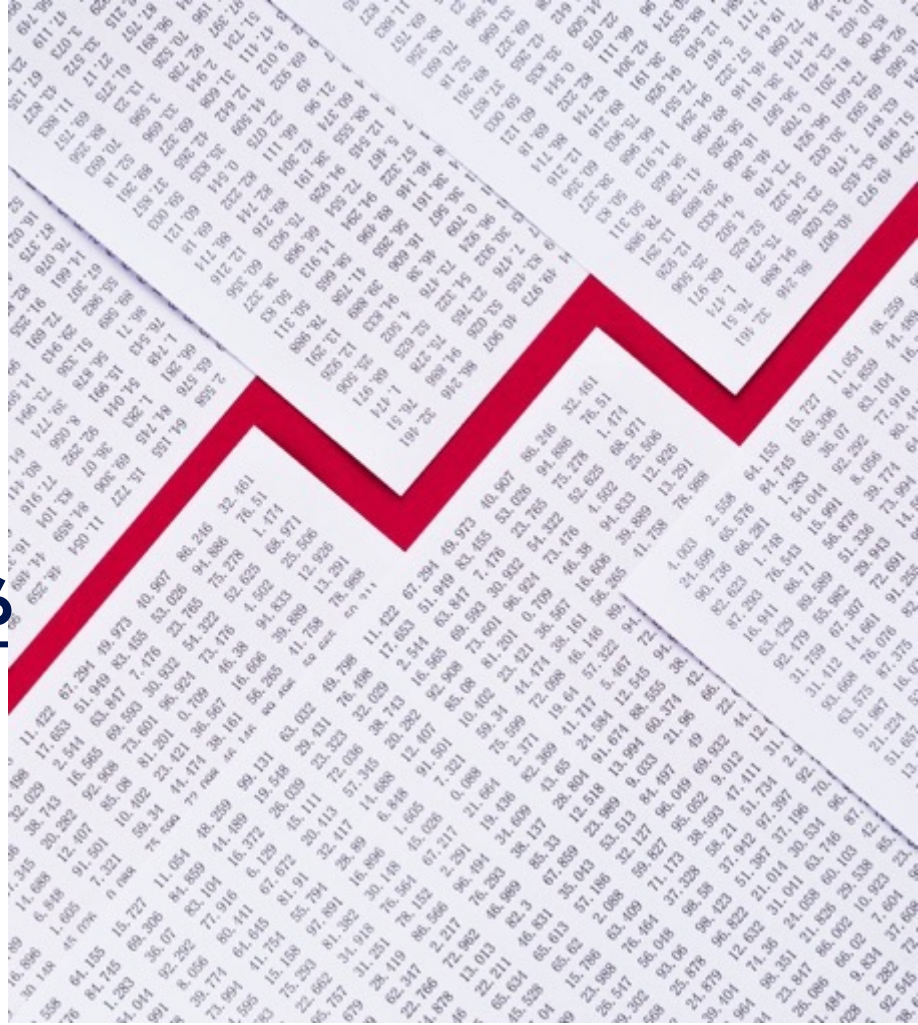
- **July 23: micro/miniVR patent – JPTO allowance**
- **Sep 9 MDL-202 patent - USPTO allowance**
- **Oct 20: MDL-101 patent – JPTO allowance**
- **Sep 11: Capsid License from Exegenesis bio**



A layered IP portfolio that covers foundational technology and product for our competitive edge

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2. Financial updates @ the end of 3Q FY2026



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BS & Financial Position at the end of 3Q/2026

The cash position decreased from the end of FY2025 due to continued investment in R&D.

(Million Yen)

	End of FY2025 (A)	3Q FY2026 (B)	(B) – (A)
Current assets	2,892	2,408	△484
Cash & deposits	2,812	2,368	△444
Non-current assets	71	50	△21
Total assets	2,964	2,459	△505
Current liabilities	134	141	7
Non-current liabilities	36	1	△35
Total liabilities	170	142	△28
Total net assets	2,793	2,317	△476
Total liabilities and net assets	2,964	2,459	△505
Capital adequacy ratio	93.0%	91.9%	

Note

- Despite continued financing through the exercise of stock acquisition rights, cash and deposits decreased from the end of FY2025 due to continued investment in R&D.

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BS & Financial Position at the end of 3Q/2026

The cash position decreased from the end of FY2025 due to continued investment in R&D.

(In thousand USD at 160yen/$)

	End of FY2025 (A)	3Q FY2026 (B)	(B) – (A)
Current assets	18,075	15,050	-3,025
Cash & deposits	17,575	14,800	-2,775
Non-current assets	443	312	-131
Total assets	18,525	15,368	-3,156
Current liabilities	837	881	43
Non-current liabilities	225	6	-218
Total liabilities	1,062	887	-175
Total net assets	17,456	14,481	-2,975
Total liabilities and net assets	18,525	15,368	-3,156
Capital adequacy ratio	93.0%	91.9%	

Note

- Despite continued financing through the exercise of stock acquisition rights, cash and deposits decreased from the end of FY2025 due to continued investment in R&D.

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PL & Business Result at the end of 3Q/2026

Operating expenses of ¥1,686 million were recorded, mainly reflecting activities to advance the MDL-101 program toward clinical trials.

(Million Yen)

	3Q FY2025 (A)	3Q FY2026 (B)	(B)−(A)
Operating revenue	-	-	-
Operating expenses	1,815	1,686	△129
R&D	1,632	1,536	△96
SGA	182	149	△33
Operating income	△1,815	△1,686	129
Ordinary income	△1,798	△1,650	148
Current Profit	△1,801	△1,608	193

Operating expenses

- The Company continued to invest in research and development as development activities centered on MDL-101 progressed.
- At the same time, the Company continued to pursue financial strategies, including capital policy initiatives and the use of external financing.

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PL & Business Result at the end of 3Q/2026

Operating expenses totaled approximately US$10.5 million, mainly reflecting activities to advance MDL-101 toward clinical trials.

(In thousand USD at @160yen/$)

	3Q FY2025 (A)	3Q FY2026 (B)	(B)−(A)
Operating revenue	-	-	-
Operating expenses	11,343	10,537	-806
R&D	10,200	9,600	-600
SGA	1,137	931	-206
Operating income	-11,343	-10,537	806
Ordinary income	-11,237	-10,312	925
Current Profit	-11,256	-10,050	1,206

Operating expenses

- The Company continued to invest in research and development as development activities centered on MDL-101 progressed.
- At the same time, the Company continued to pursue financial strategies, including capital policy initiatives and the use of external financing.

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Financial Foundation and Growth Investment Supporting MDL-101's Clinical Transition

Funding progress and reduced development risk position Modalis at a pivotal stage of value creation

Funding Progress & Financial Position

19th Series Warrants – Exercise Progress

Over 80%

Consolidated Cash & Deposits | Sep. 2026

¥2.37B

Straight Bond has been fully redeemed.
Although total proceeds are expected to fall below the original estimate due to share price levels, the Company maintains a financial base to support current operations.

Development Risk Reduction

GMP Manufacturing Completed

Product release testing ongoing

Additional GLP Toxicology Study Progressing

7 weeks completed; 3-month data to follow

Clinical Trial Readiness

Site shortlisted; contracting discussions advancing

IND Submission Preparations Advancing

Expanding Value-Creation Opportunities

MDL-101 IND Submission / FIH

A new stage of clinical validation

EMA Orphan Drug Designation (ODD) Application

Building a foundation for European development

US ODD Application

Building a foundation next gen program

Partnering & Follow-on Programs

Expanding the value of the GNDM platform

Pursuing Value Creation Through Growth Investment

19th Series Stock Options: Temporary Suspension of Exercise to Take into Account Capital Efficiency and Stock Supply and Demand → Please refer to Q&A

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3. Where we are and the Growth Strategy



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How MDL-101 Paves the Way for Follow-on Programs

Shared development assets built through MDL-101 can accelerate follow-on muscle disease programs

Gateway to Growth

MDL-101

LAMA2-RD
Ultra-rare muscle disease

US 1,000~2,500 pts

Building expertise in nonclinical development, CMC, regulatory affairs and clinical trial readiness

Moving into Clinical Validation

Five Development Assets for Follow-on Programs

1. **GNDM(dCas9) Safety Data**
   Early safety assessment of shared technology
2. **AAV CMC & Manufacturing Know-how**
   Reuse of manufacturing, QC and analytical expertise
3. **FDA Regulatory & IND Experience**
   Transferable regulatory interaction and IND preparation know-how
4. **Muscle Disease Clinical Infrastructure**
   Leveraging sites, endpoints and trial operations
5. **Clinical Validation of GNDM in Humans**
   Safety, target engagement and efficacy yet to be evaluated

1–4: Assets being established | **5: Future validation**

Expansion into Larger Muscle Disease Markets

Lower shared risks and improve development efficiency

MDL-201 DMD	~200,000 patients
MDL-202 DM1	~90,000 patients
MDL-103 FSHD	~150,000 patients

Leveraging MDL-101 experience to address larger patient populations

Longer-term Growth

CNS: Establishing BBB-penetrating delivery through collaboration with JCR and others could unlock new GNDM indications

Investment in MDL-101 builds not only a single-disease program, but also a shared development foundation for follow-on programs

Note: Follow-on programs require target- and capsid-specific validation. Patient estimates are based on [illegible]

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4. Summary



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Key Takeaway of 3Q/2026 report

1. Analysis of the IND enabling study is complete. The study confirmed a **significant improvement** in **survival rates** and **functional recovery** following administration, backed by the expression of the **LAMA1 protein** in muscle tissue due to GNDM expression.
2. **Additional GLP-tox studies** in NHP are currently underway. The study is progressing toward a 3-month readout in Dec.
3. **GMP manufacturing** is complete. Secured the quantity required for the FIH trial. Release testing is currently underway in preparation for a Dec release.
4. Significant progress in **IP**. Received a JPTO's allowance for **10**1 and for the core GNDM technology (**microVR**) patent, a USPTO's allowance for **202** and obtained a license from Exegenesis Bio for the muscle-targeted capsid patent for **201**.
5. Exercise rate for the **19th series** of SO rights exceeded **80%.➔** to **pause the exercise**

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Modalis Therapeutics



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- Based in Greater Boston area
- Pioneering the first CRISPR-based gene modulation technology since 2016
- Leading company in CRISPR epigenetic modulation
- Develops novel precision medicines for genetic disorders that have no cure



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5. Q&A



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Q1. What remains to be completed before the MDL-101 IND submission?

- Major development activities required for the MDL-101 IND are progressing well. Mouse IND-enabling studies and GMP manufacturing have been completed. Product release testing is now underway, and the additional GLP toxicology study is progressing.
- Key remaining milestones include obtaining and evaluating the **three-month GLP toxicology data**, completing **GMP product release testing**, and integrating these results into the **IND submission package**.

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Q2. Has the issue encountered in the previous GLP toxicology study been resolved?

- **Yes**. In the previous GLP toxicology study, transduction to the target tissues was insufficient, making it difficult to properly evaluate the investigational product.
- Subsequent investigations suggested that the **pre-dose immune pretreatment regimen**, rather than the test article itself, may have affected transduction efficiency.
- Following optimization of the pretreatment protocol, additional nonclinical studies confirmed target-tissue transduction and increased LAMA1 expression.
- The ongoing additional GLP toxicology study has also progressed generally well through seven weeks after dosing. We are proceeding toward collection and evaluation of the planned three-month data.

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Q3. Has GMP manufacturing secured sufficient clinical supply and product quality?

- GMP manufacturing of the investigational product has been **completed**, with the manufacturing process progressing largely as planned.
- Based on preliminary manufacturing assessments, we believe that **sufficient product quantity has been secured** for the planned clinical trial.
- Quality-related release testing is ongoing, with **product release targeted for December 2026.** Final quality compliance will be determined based on the release test results.

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Q4. How confident are you in the IND submission target of late 2026 to Q1 2027?

- We continue to target IND submission between **late 2026 and the first quarter of 2027**.
- The nonclinical, CMC, and clinical-readiness workstreams required for the IND are making steady progress, increasing our confidence in the feasibility of the plan.
- However, the submission timing remains subject to the forthcoming GLP toxicology data, quality-testing results, and interactions with regulatory authorities. The timeline is therefore not guaranteed.

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Q5. What would constitute success in the MDL-101 clinical trial?

- The primary priority in the first-in-human study will be to assess **safety and tolerability**.
- It will also be important to determine whether GNDM produces its **intended effects in humans**, including increased LAMA1 expression in muscle tissue.
- We will also assess clinical measures such as muscle function and seek early signals of potential efficacy. Demonstrating **target engagement** in humans would be a critical validation not only for MDL-101 but also for the broader GNDM platform.

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Q6. How far has clinical-site selection progressed, and when could the first patient be dosed?

- Selection of the U.S. clinical trial site and principal investigator (PI) has been **substantially completed**.
- We have established a close working relationship with the institution through joint regulatory activities and are advancing preparations for trial execution. Formal contracting and other procedures will follow.
- We aim to initiate first-patient dosing promptly once the IND becomes effective and site activation and patient enrollment requirements are met.

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Q7. Will lower-than-planned proceeds from the 19th Series warrants affect development?

- The 19th round of fundraising through stock options is largely on track, with the conversion of corporate bonds now complete and the exercise rate of stock options exceeding 80 percent.
- While the total amount raised may fall short of initial projections due to stock price levels, the consolidated cash and deposit balance as of the end of September 2026 is estimated to be approximately 2.37 billion yen, **ensuring the financial foundation necessary for immediate business operations, including the clinical transition of MDL-101.**
- We are temporarily suspending the exercise of stock options at this time; however, this is not due to a shortage of funds, but rather a **decision made after comprehensively considering fundraising efficiency, as well as the impact on stock supply and demand and dilution**.
- We will continue to prioritize the transition of MDL-101 to clinical trials and allocate funds efficiently in accordance with our development plan.

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Q8. Could additional financing or shareholder dilution occur in the future?

- In addition to progress in the clinical development of MDL-101, we view the development of follow-on muscle disease programs targeting larger markets as a key growth opportunity.
- We have several key development milestones scheduled for the future, and **we expect that progress toward these milestones will further enhance the market's understanding and valuation of our business.**
- Taking these business developments, the market environment, and stock price levels into comprehensive consideration, **we plan to raise capital at the appropriate times while prioritizing capital efficiency.**
- In addition to the efficient use of our cash on hand, we aim to enhance corporate value by utilizing multiple options, such as securing funds through business partnerships and raising capital via the capital markets.

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Q9. Please explain the reasons for the temporary suspension of the exercise of the 19th series of stock options and the plan for resuming them in the future.

- After comprehensively considering current stock price levels, supply and demand for shares, market conditions, and other factors, **we have decided to temporarily suspend the exercise of the 19th Series Stock Subscription Rights in order to appropriately manage the efficiency of our capital raising and the impact of dilution on existing shareholders.**
- At this time, **the suspension period is set to last until December 18, 2026**; however, this does not represent a fixed capital raising schedule. We reserve the right to extend or move up this schedule with five(5) business days' notice. We will issue an announcement if the suspension period is changed.
- Several important development milestones, centered on MDL-101, are scheduled for the future. We will comprehensively consider the progress of these milestones, market conditions, stock price levels, and other factors, and **will review the suspension period and the timing of the resumption of exercise as necessary.**
- The Company has secured the funds necessary for its immediate business operations, and **our policy is to utilize the remaining stock options with a focus on corporate value and capital efficiency, rather than prioritizing early fundraising.**

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Q10. How much can MDL-101 reduce development risks for MDL-201, MDL-202, and MDL-103?

- MDL-101 is both a standalone development program and a lead program that establishes a development foundation for follow-on programs.
- We are accumulating **reusable assets and know-how**, including dCas9 safety assessment, AAV manufacturing and quality control, regulatory interactions, and a clinical-development framework for muscle diseases.
- Applying these insights to follow-on programs could reduce shared development risks and improve efficiency. Program-specific efficacy and delivery technologies will nevertheless require separate validation.

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Q11. What milestones beyond MDL-101 should investors watch?

- Alongside the **MDL-101 IND submission** and clinical transition, we are preparing an application for **Orphan Drug Designation in Europe** (EMA ODD) for MDL-101 and **US ODDs** for the other programs.
- In addition, under our subsequent neuromuscular disease programs, we are advancing the development of each program, including nonclinical validation of MDL-201 and the submission of an **US orphan drug designation application** (which has been completed).
- From a business-development perspective, we continue to explore partnering opportunities with pharmaceutical companies. We are also evaluating future expansion into CNS diseases through BBB-penetrating delivery technologies, including our collaboration with **JCR Pharmaceuticals**.

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Q12. Is Anthropic's entry into LAMA2-RD research a competitive threat or an opportunity?

- We welcome Anthropic's entry into LAMA2-RD research as evidence of increasing interest and new R&D investment in this rare disease.
- The approach announced by the company involves using **bispecific antibodies** to strengthen the bond between muscle fibers and the basement membrane; its mechanism of action differs from that of MDL-101, which activates LAMA1 expression and **not only strengthens the bond but also produces a cross-linking** effect.
- The programs are currently at different development stages, although they could eventually compete. At the same time, progress across different mechanisms may deepen understanding of LAMA2-RD and expand treatment options.

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Q13. How are you addressing Internet security issues, including the recent PHP vulnerability?

- We recognize information security as a critical management issue in our business operations and are taking measures to address it in collaboration with external specialized service providers.
- Regarding the recent PHP vulnerability issue, we have confirmed that our website is affected and have taken temporary measures to take the site offline in order to block external access. According to the findings of our contracted service provider, no suspicious processes or other issues have been reported at this time.
- Please note that **research and development data and confidential information are managed separately in a cloud environment independent of the website in question, equipped with access controls and other security measures, and are thus isolated from the management environment affected by this website vulnerability.**
- We are currently discussing with our contractor and considering updates to the website infrastructure, including the server, operating system, and PHP. We remain committed to maintaining and improving the necessary security measures and management systems.

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