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In case of any discrepancy, the Japanese version shall prevail

The First Quarter of 2026
Business and Financial report

(TSE : 4883)

Modalis therapeutics Corporation

May 13th, 2026

MODALIS

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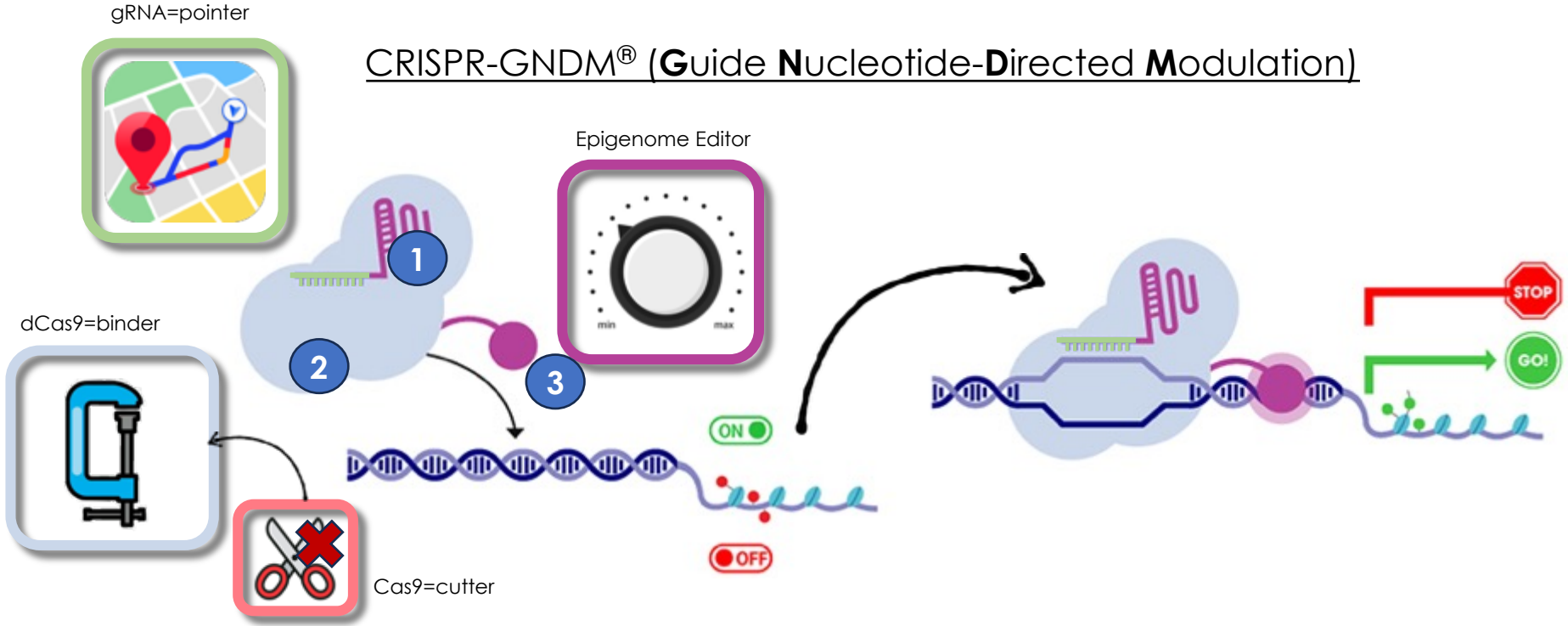
About Modalis

MODALIS Value Highlights

- Established a robust epigenetic editing platform for activation and inhibition of endogenous genes using CRISPR-GNDM®
- Demonstrated sustained modulation of gene expression across species resulting in functional **efficacy while maintaining an encouraging safety profile**
- Pipelines in **muscular dystrophies**, additional programs in CNS, cardiovascular and broad therapeutic potential
- **Manufacturing process established** for challenging AAV capsids to enable tissue tropic delivery for programs
- **Experienced team** with deep knowledge of platform
- Strong **IP portfolio and strategy** that includes granted and licensed patents
- Regulatory engagement supporting refinement of the clinical development strategy

CRISPR-GNDM®

GNDM controls the expression of target proteins through epigenetic regulation



CRISPR-GNDM® consists of: ① a "guide RNA (gRNA)" for positioning, ② 'dCas9' as the binding device to DNA, ③ an "editor" for editing the epigenome, enabling precise control over gene expression on and off.

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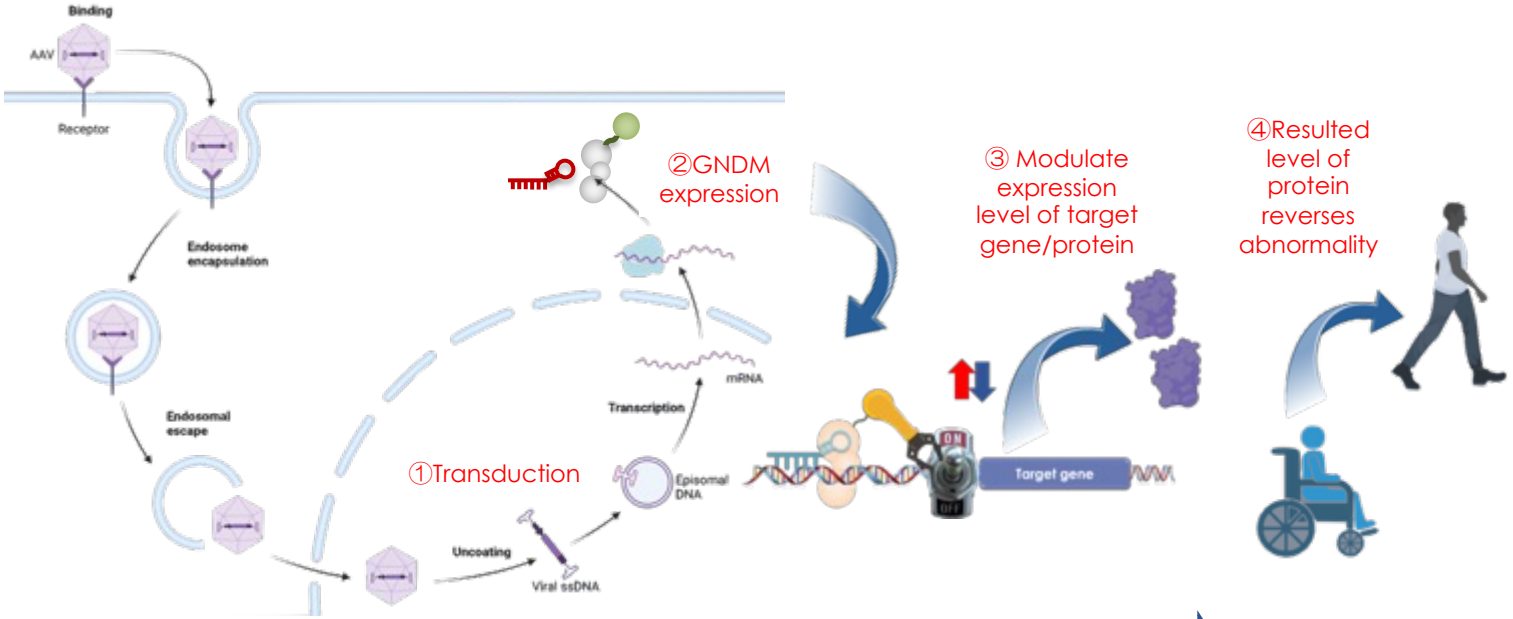
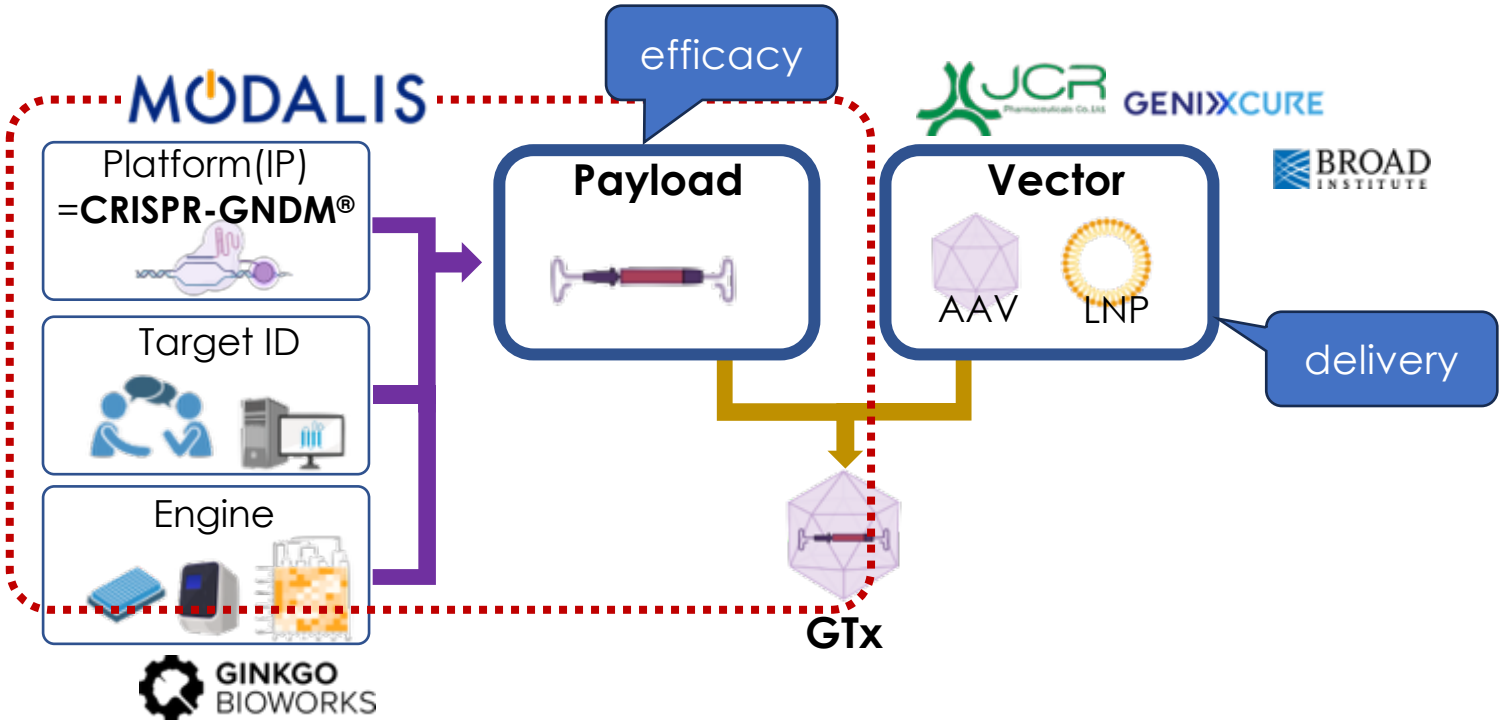
Delivery of GNDM to the target

Packed in a vector, GNDM is delivered to target cells and selectively controls the expression of the target gene



MODALIS' core competence and collaboration

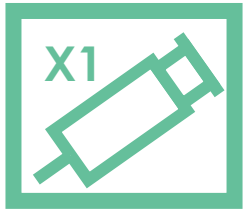
For GTx, the payload—the active component—and the cargo (vector) that delivers it to the target tissue are crucial



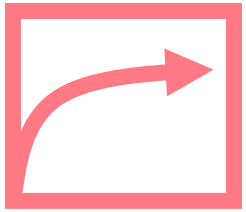
CRISPR-GNDM®'s advantages

A single injection provides long term disease modifying effect

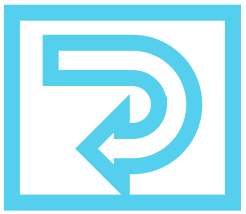
Potential benefits of CRISPR-GNDM® Technology



Single dose
Doesn't require
Repeated dosing



Long-lasting
Sustained effect
for years or decades



Disease Modifying
Not just to reduces
symptoms but
aims to provide
transformative
therapeutic benefit

Updated

Approval Status of GTx

Otarmeni became the second in vivo gene therapy approved in 2026

Gene therapies approved by US FDA

Trade Name	Year of Approval	cost	Indication	Manufacturer	Patient Population	WW market size* (mil USD)
Lxturna	2017	$850k	RPE65	Roche(Spark)	2 per 100,000	$65M
Zolgensma	2018	$2.1M	SMA	Novartis(Avexis)	1 in 15,000 live births (Approx. 100s ~1000 patients in US)	$1.3B[#3]
HEMGENIX	2022	$3.5M	Hemophilia B	uniQure CSL Behring	1 in 30,000 male	$88M[#3]
Vyjuvek	2023	$631k per patient year	DEB[*2]	Krystal	3.5–20.4 in 1 million	~$200M[#2]
Roctavian		$2.9M	Hemophilia A	BioMarin	1 in 5,000 male	$262M[#4]
beqvez	2024	$3.5M	Hemophilia B	pfizer	1 in 30,000 male	$88M[#3]
ELEVIDYS		$3.2M	DMD	Sarepta	1 in 3,500 male birth	$4.1B[#4]
Itvisma	2025	$2.6M	SMA	Novartis	Over 2yrs SMA patients Approx. 1000s ~10k patients in US	~$10B[#2]
Kresladi	2026	$2~3M?	**LAD-I**[*4]	Rocket	1 in million (25 pts/yr・WW)	$25M~50M
Otarmeni		TBD	hearing loss	Regeneron	50 newborn in a year/US	$130 million

Just approved

Source: National Organization for Rare Disorder、#2 Fierce Biotech #3 Corporate website #4Grand view research #5 Fortune Business Insight

*1: Spinal muscular atrophy *2: dystrophic epidermolysis bullosa *3: Duchenne muscular dystrophy *4: severe leukocyte adhesion deficiency type I

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Epigenome editing competitive landscape

The field is gaining more traction and MODALIS is uniquely differentiated from the other epigenome companies

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 B ($68M, 2025)	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	**SerC? ($75M, 2025)**	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**

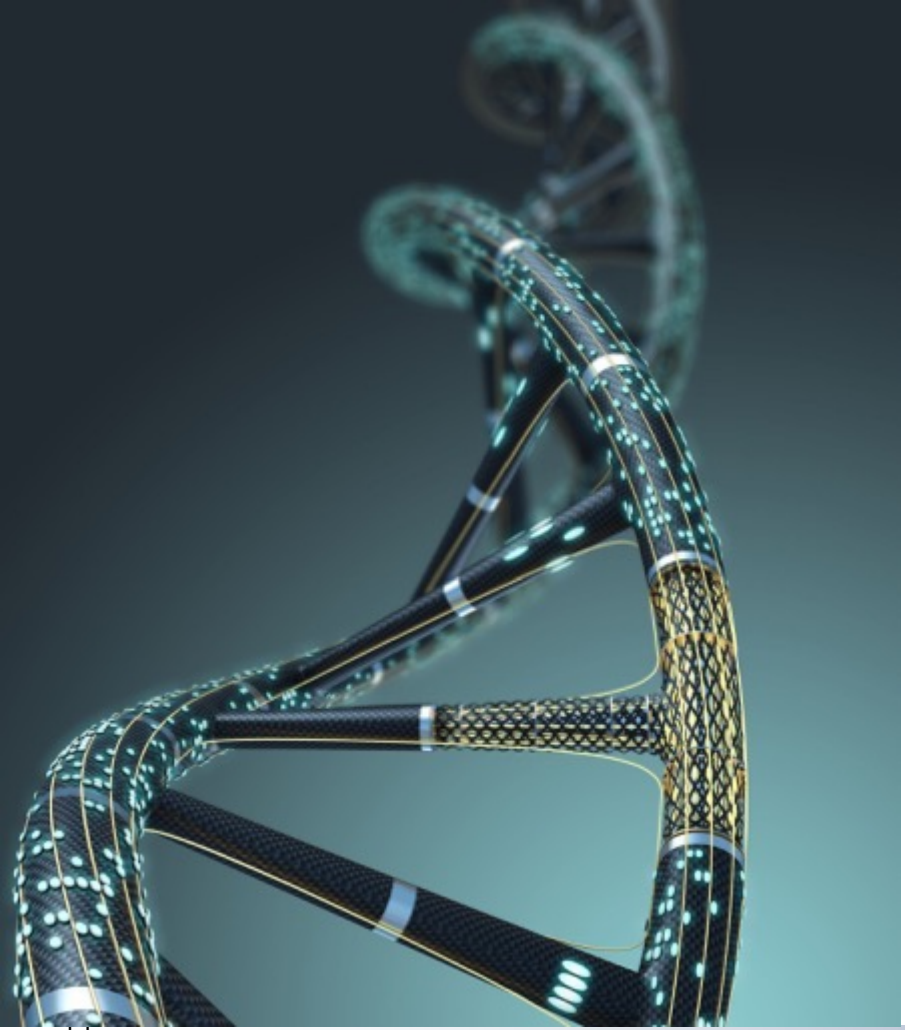


Table of contents

1. Business Highlights of the 1Q/2026

01

MDL-101 Progress and Timeline

02

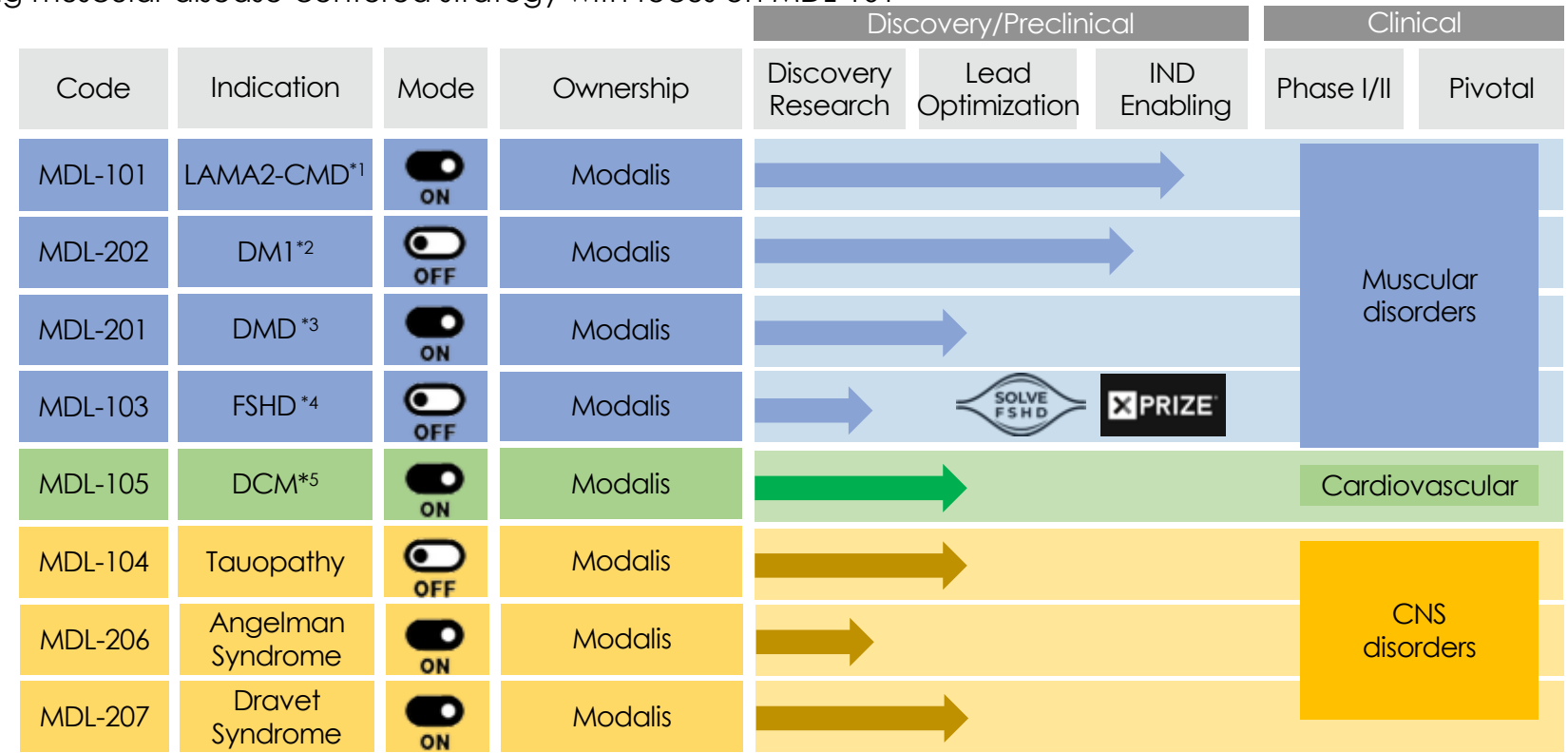
Progress in the other programs

03

The other progress in the business

The current pipeline of MODALIS

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



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

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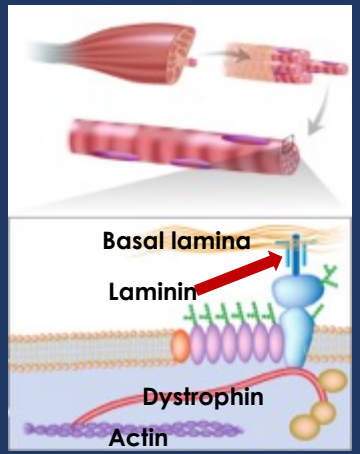
LAMA2-CMD (aka CMD1a)

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

MDL-101

Potential to be the first LAMA2-CMD gene activation therapy



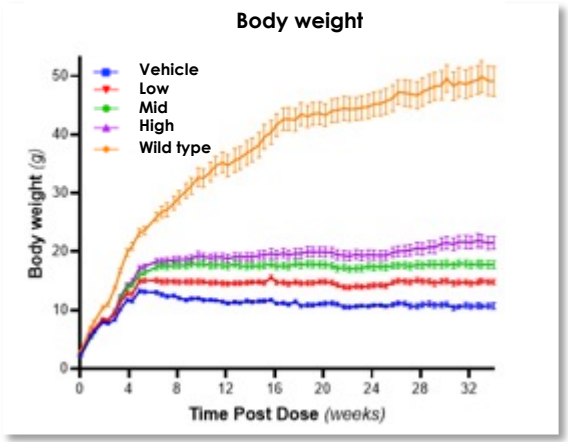
Prevalence	**8.3 in 1 million*** 2,500 in US	
Disease Onset	Apparent at birth or within a few months after birth	
Disease Burden	Patients do not survive past adolescence	• Severe muscle weakness • Lack of muscle tone (hypotonia) • Little spontaneous movement • Joint deformities (contractures) • Heart problems and seizures
Disease Causing Gene	**LAMA2 mutation**	
Commercial opportunity	**$500M+**	

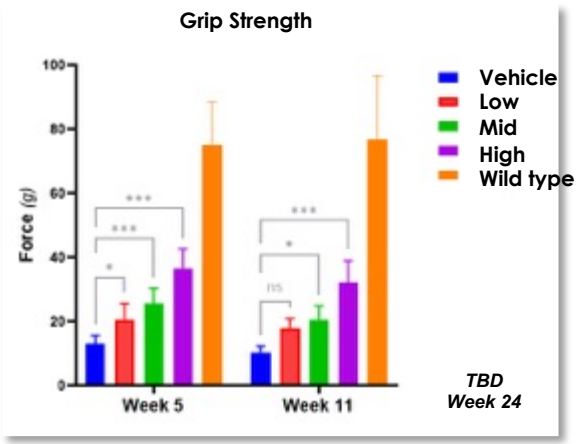
Source: *Estimating the Prevalence of LAMA2 Congenital Muscular Dystrophy using Population Genetic Databases (2023)

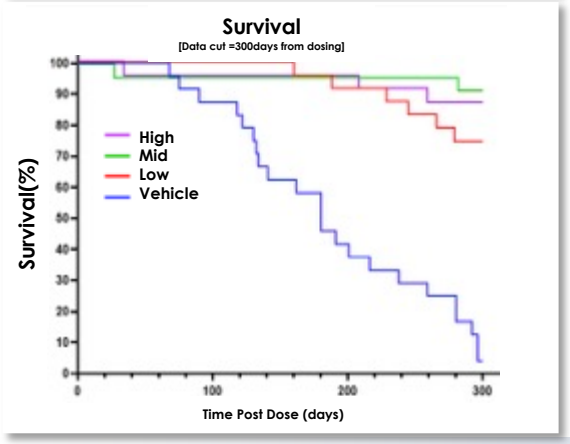
Improvements in body weight, grip strength, and survival in disease model mice

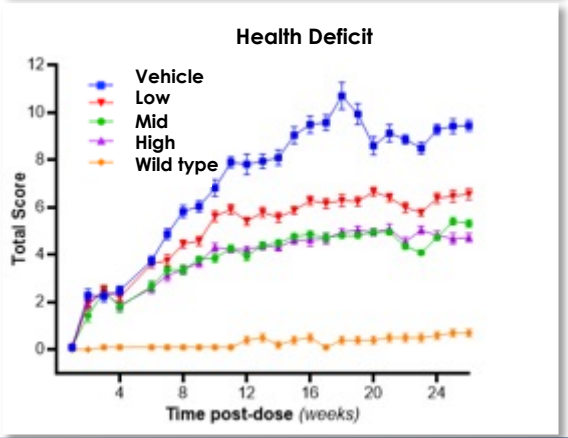
Administration of mMDL-101 significantly improved the clinical condition in patients with LAMA2-RD

Updated









Mouse Health Deficit Assessment - *Overview*

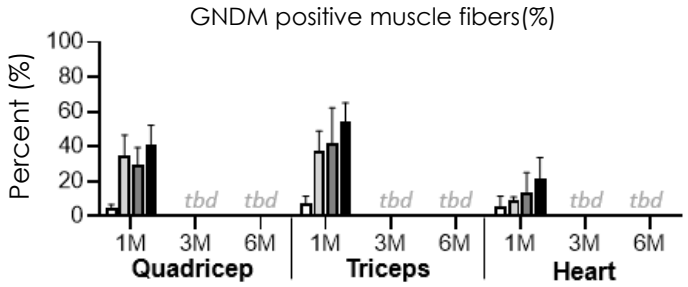
(9-parameter, 3 points scale)

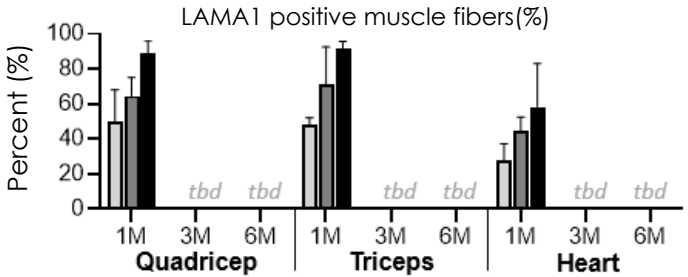
		Score			
	Parameter	0	1	2	3
1	Activity	Normal	Mild deficit	Deficit	Severe deficit
2	Provoked behavior				
3	Locomotion				
4	Respiration				
5	Posture				
6	Body condition				
7	Fur and skin				
8	Eyes				
9	Tumors & infections				

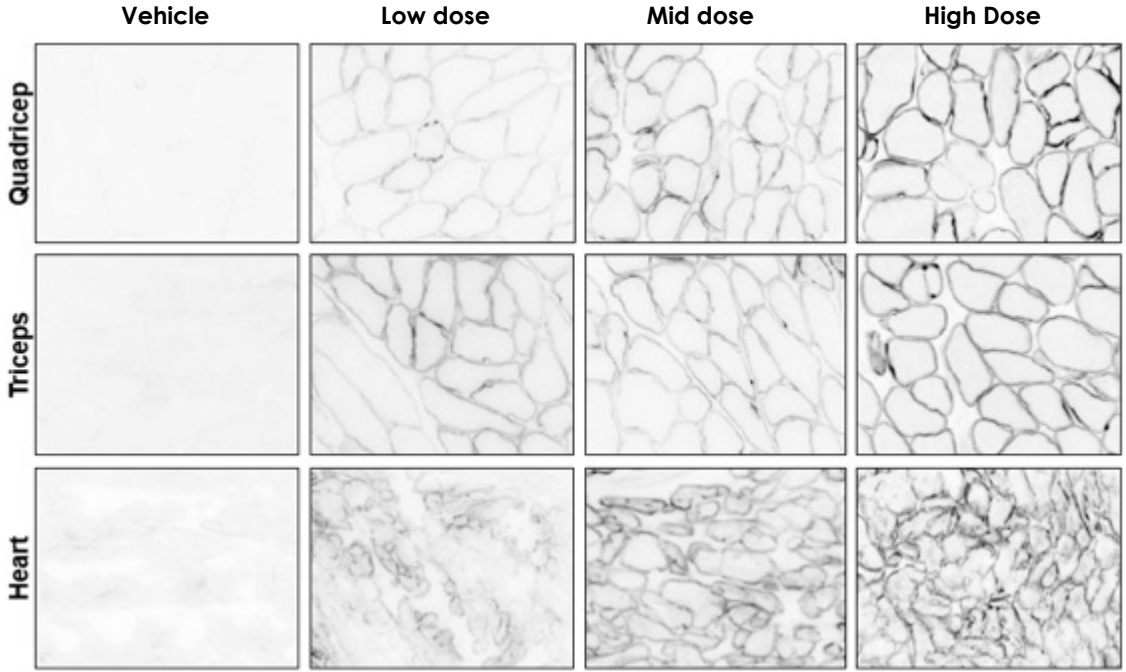
LAMA1 distribution by MDL-101

Robust, Dose-dependent LAMA1 protein induction in Quad, Triceps, and Heart via GNDM protein expression

Updated





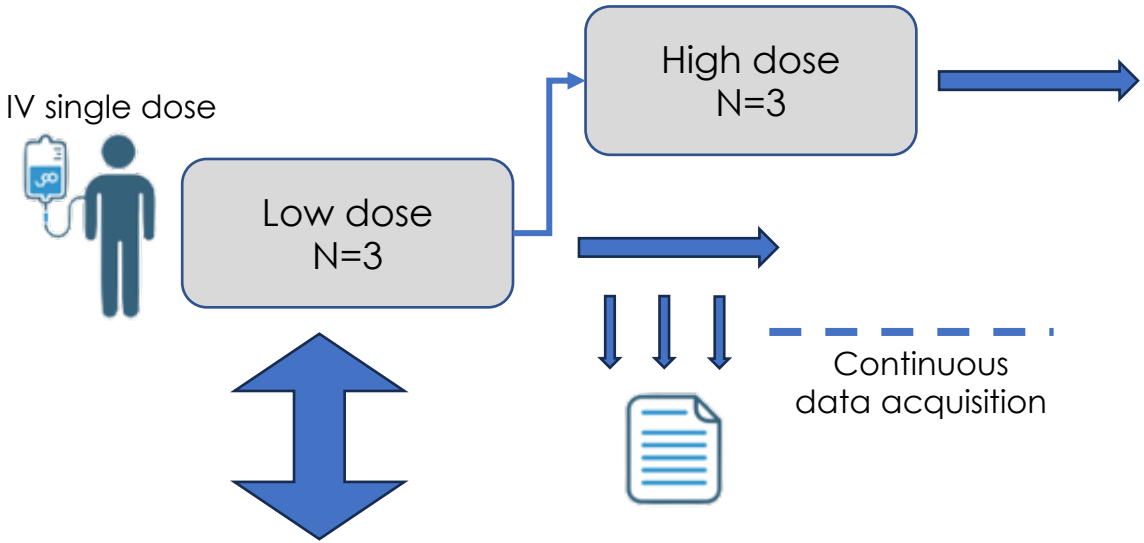


MDL-101-001 Trial design

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

- Patients aged 36 months or younger (male or female)
- Clinical condition and/or significant reduction in LAMA2 protein levels in muscles associated with Lama2 gene mutations
- Stable condition during treatment
- Difficulty with independent walking or sitting

Phase1/2 Open-label dose escalating trial



Compare with Natural History Study
(NCT06354790, NCT04299321, NCT06132750)

Progress towards the FIH trial

Development update of MDL-101

- GLP Toxicity Study
  - GLP toxicity studies are ongoing, and we are currently conducting additional analyses and optimization studies to maximize the likelihood of long-term clinical success. At this time, no new significant safety signals have been identified.
- Mouse IND Enabling
  - The study is progressing smoothly. In ongoing analyses, the study has demonstrated significant improvements in survival and functional recovery compared to the control group in the disease model and is expected to be completed without issues during this quarter.
- Manufacturing
  - Awaiting GMP manufacturing of the AAV investigational drug. Pilot production at GMP scale has been completed.
- Clinical Trial Preparation
  - Currently selecting clinical trial sites and principal investigators.
  - Preparing for various evaluation studies.
  - Re-evaluating the development timeline to maximize the probability of a successful transition to clinical trials.

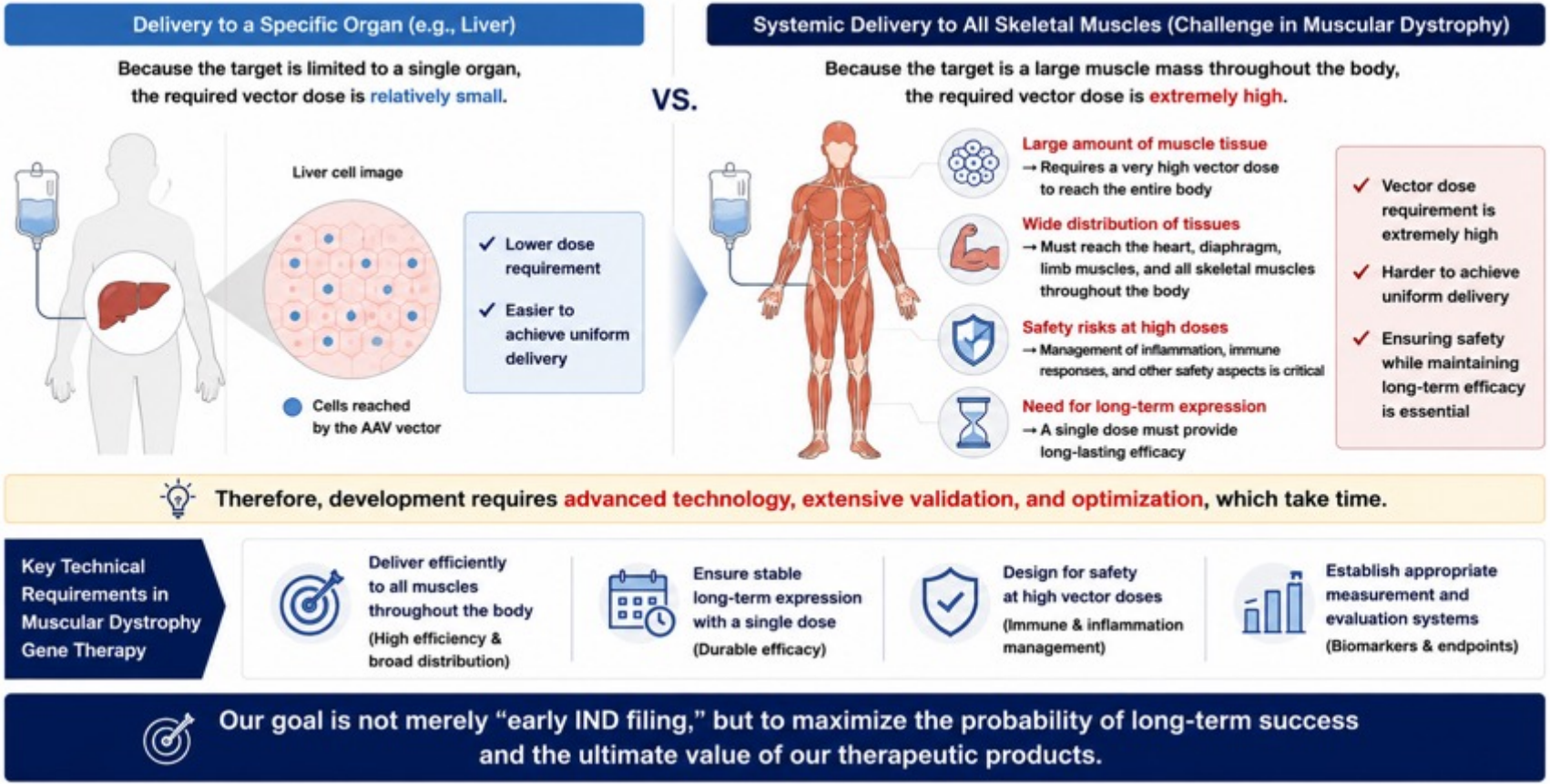
Current Priority Initiatives

The "Last Mile" Toward Clinical Translation

- ✅ Strengthening Manufacturing and Quality Assessment
  - Optimization of manufacturing processes and quality assessment systems with a view to future clinical development and commercialization
- ✅ Long-term Expression Profile Analysis
  - Detailed evaluation of expression persistence and long-term efficacy in target tissues
- ✅ Optimization of Dosage Strategies
  - Optimization of dosing conditions and immunosuppression protocols with a view to clinical trials
- ✅ Preparation for Clinical Transition
  - Coordination of evaluation metrics and measurement timelines with clinical trial sites, and establishment of a clinical trial operational framework

Why does development take time?

In the field of muscular dystrophy, achieving systemic delivery, long-term expression and safety requires extensive technical validation and optimization



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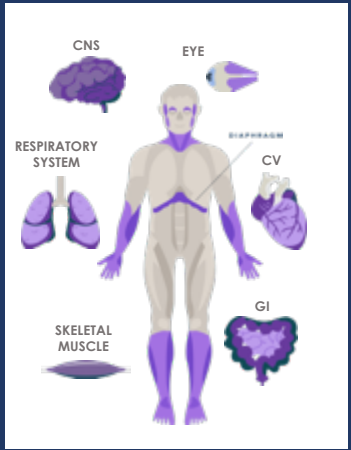
Myotonic dystrophy type 1(DM1)

extension of CTG repeat in 3' UTR of DMPK gene

MDL-202

Potential to be the first-in-class and the first DM1 treatment



Prevalence	**1-4.8 in 10,000** (1 in 2,300*)	DM is the most common muscular dystrophy among adults of European ancestry
Disease onset	DM1 can occur from birth to old age	Age at onset is between 20 and 70 years (typically onset occurs after age 40)
Disease Burden	muscle weakness and wasting (atrophy), myotonia	DM causes weakness of the voluntary muscles, although the degree of weakness and the muscles most affected vary greatly according to the type of DM and the age of the person with the disorder
Cause of disease	Microsatellite expansion in 3' UTR of DMPK gene	Extended CTG repeat capture MBNL1 protein which is essential for normal splicing
Market size	**$2.2B #** By 2032	$80M market as of 2022 without any treatment but is expected grow

*Source: Myotonic Disease Foundation # DelveInsight (including both DM1 and DM2)

Molecular Pathogenesis of DM1 and MDL-202 mechanism of action

An abnormally elongated CUG traps splicing proteins and impairs their function

What is DM1?

In DM1 patients, abnormal expansion of CUG repeats in the DMPK gene causes myotonic dystrophy type 1.

What causes it?

Expanded CUG repeats in the DMPK gene produce toxic RNA that binds and sequesters MBNL proteins.
This leads to widespread splicing defects in many genes, triggering various organ dysfunction including muscles.

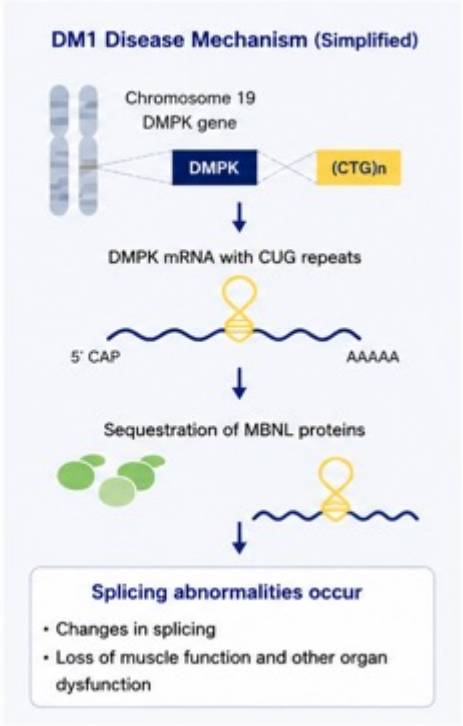
CUG Repeat Length and Disease Severity (Examples)

50–150	50–1,000	>800	>1,000
Associated with mild or non-disease (one-allele carriers)	Associated with classic or adult-onset DM1	Associated with juvenile-onset DM1	Associated with congenital-onset DM1

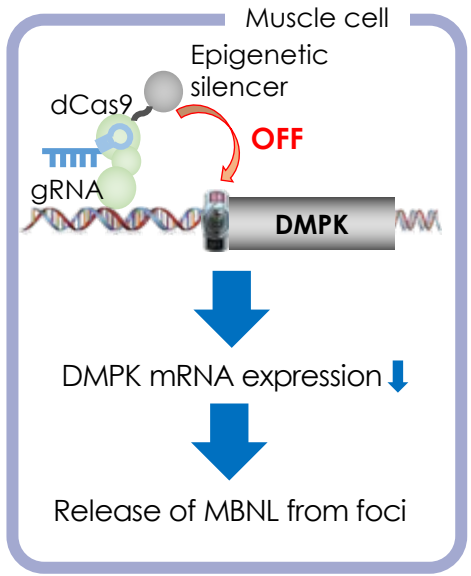
Longer repeats → Earlier onset and more severe disease

What happens as a result?

Toxic RNA binds to MBNL proteins and reduces their function.
This causes splicing abnormalities across a wide range of genes, leading to muscle dysfunction and other symptoms.



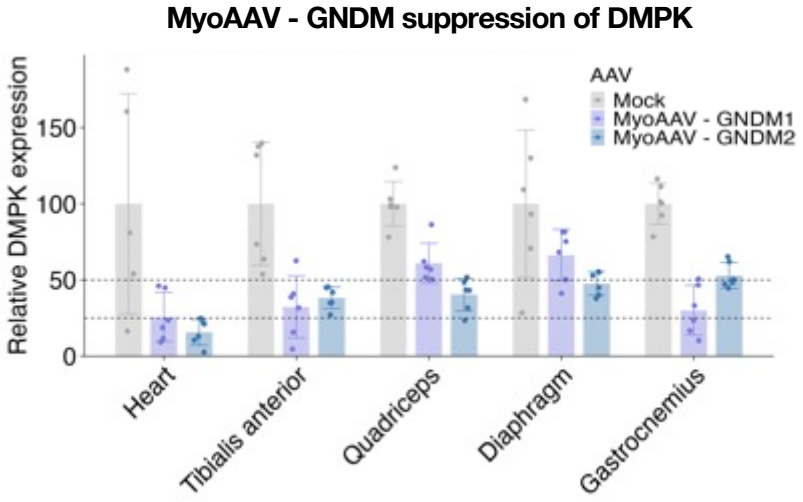
CRISPR-GNDM® targeting DMPK



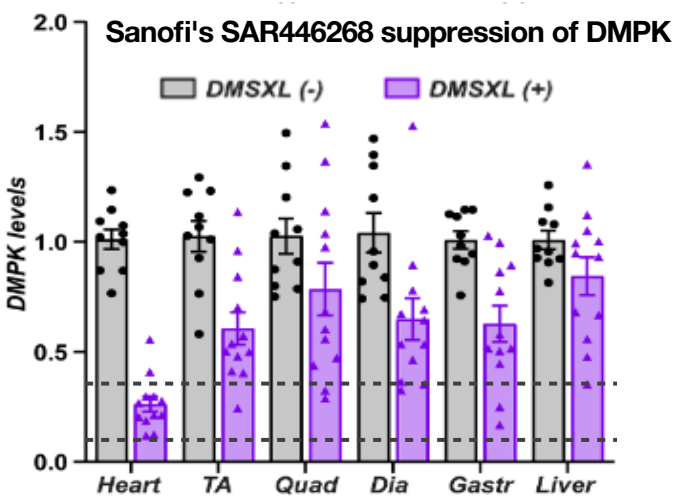
By targeting the root cause of DM1 and restoring normal splicing, we aim to improve muscle function and patients' quality of life.

Comparative evaluation of GNDM against other modalities

GNDM exhibited a greater DMPK suppression compared to vectorized RNA in all muscle tissues



8 wks dosing at 1e14 vg/kg to DMSXL mouse model (n=6)
8 wks post-injection harvest



8 wks dosing at 9e13 vg/kg to DMSXL mouse model (n=10-12)
8 wks post-injection harvest (Tomassy et al., 2025)

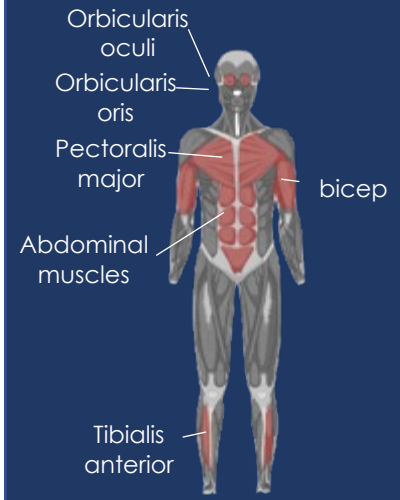
Estimated %DMPK suppression	Heart	Diaphragm	Quadriceps	Tibialis Anterior	Gastrocnemius
GNDM2-MyoAAV	87%	52%	60%	63%	49%
Sanofi '268	~76%	~32%	~25%	~40%	~35%

Facioscapulohumeral Muscular Dystrophy (FSHD)

A type of muscular dystrophy caused by impaired Dux4 gene expression

MDL-103

Potentially first-in-class treatment by silencing expression of toxic Dux4 gene product



Prevalence	**1 in 10,000-20,000**	Muscular dystrophy most frequent in adults
Disease Onset	Often not recognized until the 20s and tends to worsen during adolescence	Progression of disease to face, shoulders, and arms is generally slow
Disease Burden	weakness of the facial muscles, the stabilizers of the scapula, or the dorsiflexors of the foot	Symptoms of asymmetrical (unbalanced) muscle weakness Visual impairment, vascular abnormalities, hearing impairment, etc.
Disease Causing Gene	Over expression of Dux4 gene	DUX4 is originally expressed in germline cells but need to be suppressed in somatic cells
Commercial opportunity	**$500M+**	

Source: https://doi.org/10.1212/WNL.0000000000011425
Orphanet, Raymond A. Huml MD A concise guide

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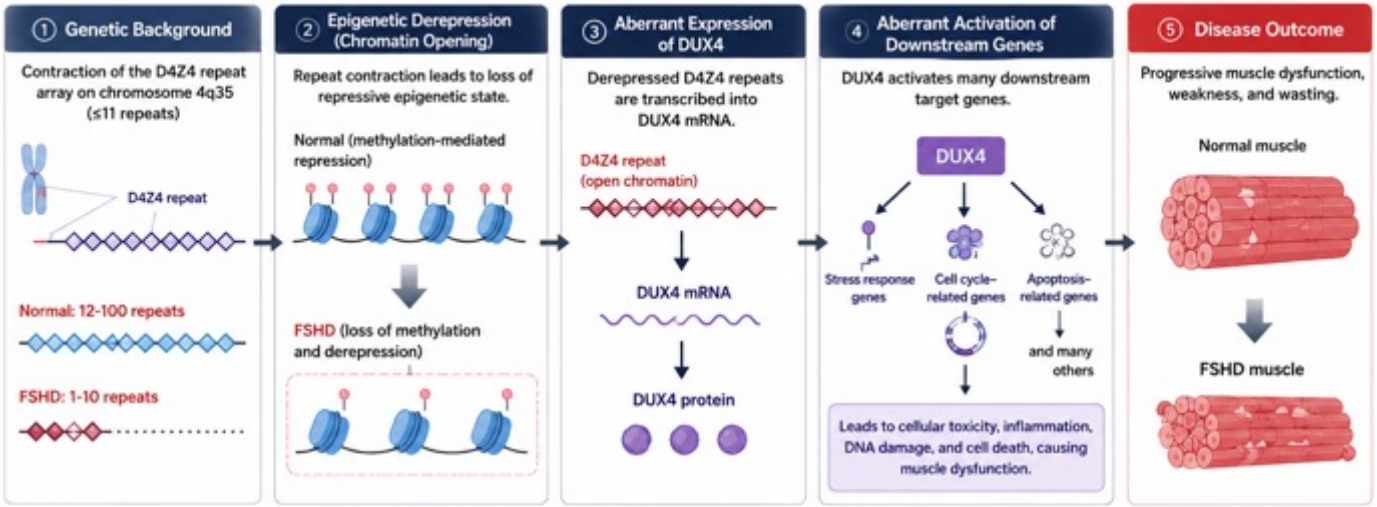
FSHD disease mechanism

Aberrant expression of DUX4 drives muscle cell dysfunction and degeneration



*This schematic diagram is based on published literature and is for illustrative purposes only.

Mechanism of action II GNDM locks Dux4

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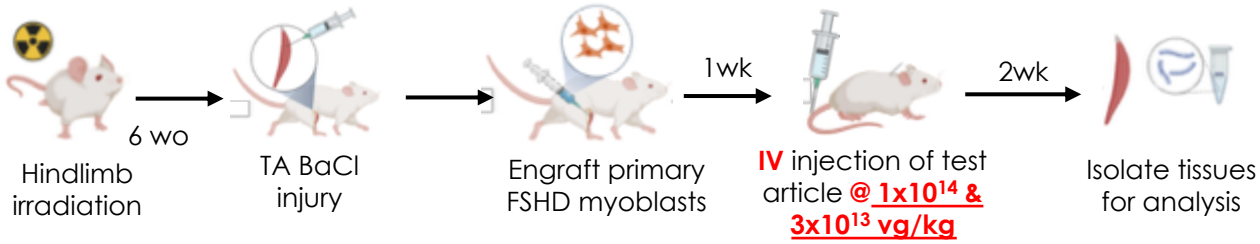
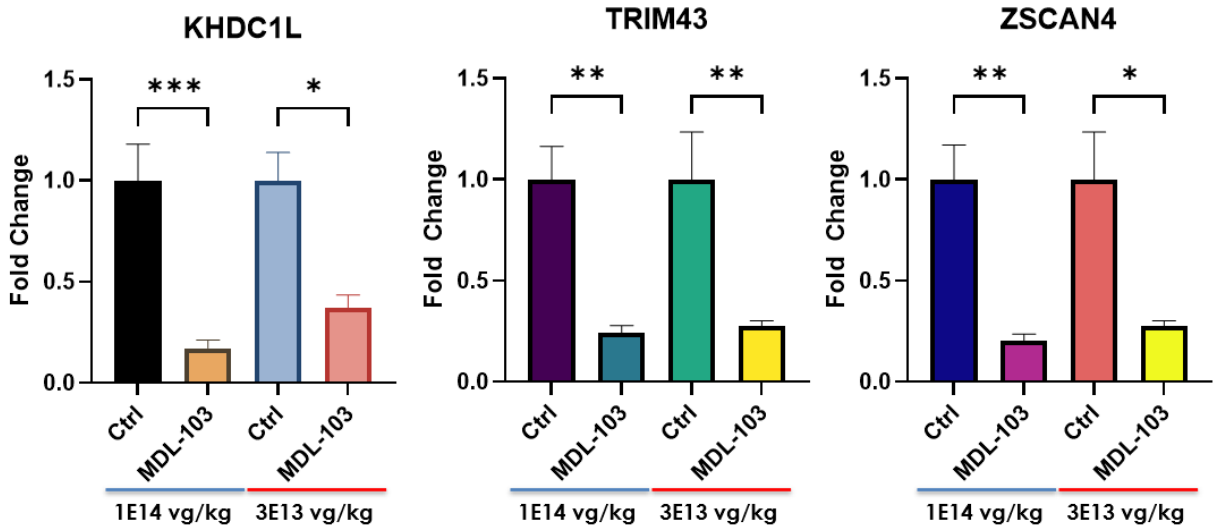
How exactly did it work in a disease model?

Dux4 downstream genes were suppressed by 70–80% by MDL-103 Systemic Administration at 3e14vg/kg



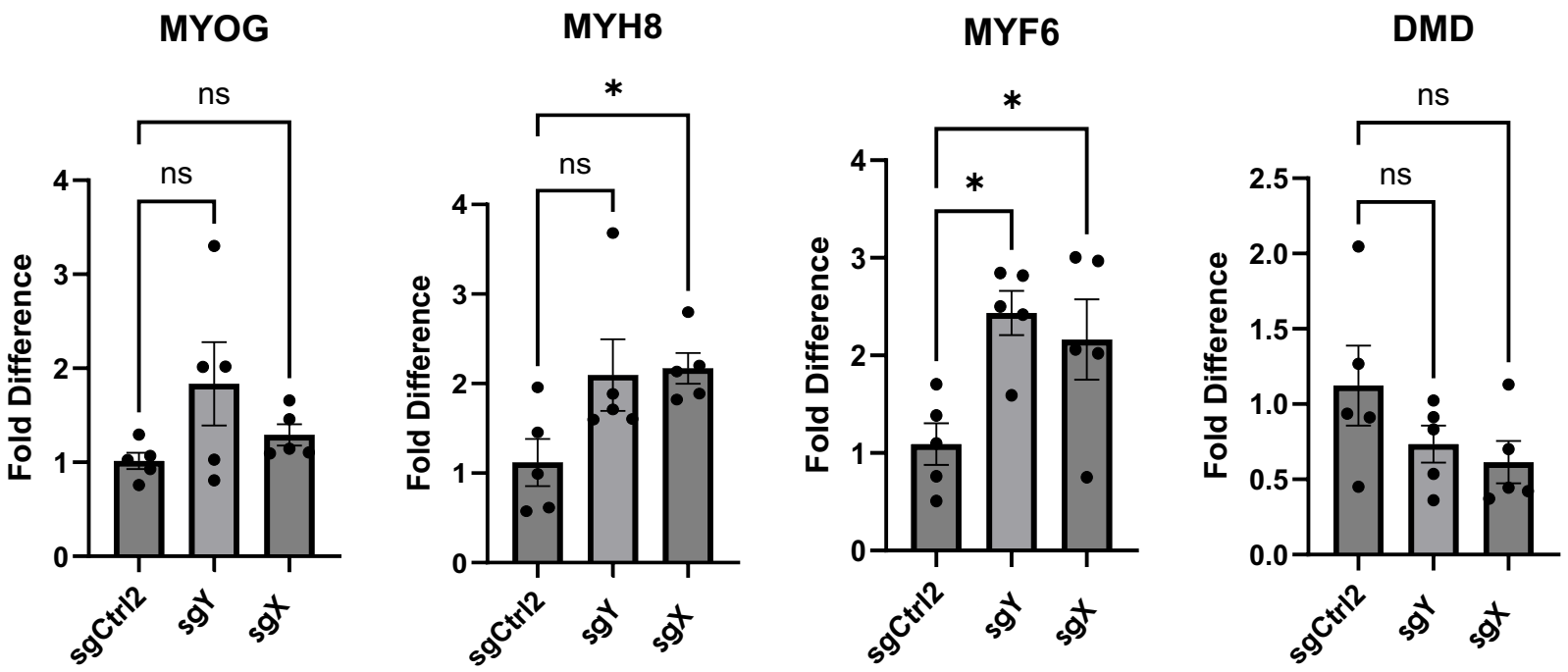


70-80% suppression of all three DUX4-target genes was achieved with MDL-103 at the even lower dose

N=5 Normalized to hRPL13A
Significance by ANOVA with Tukey's test

Targeted DUX4 repression

DUX4 suppression without compromising muscle formation



Dose: 1E14 vg/kg
Statistical significance by ANOVA with Dunnett's test

Data presentations at ASGCT and SolveFSHD conference

Solve FSHD

- Progress update of MDL-103
- Day&Time April 8th

ASGCT

- Title: Pairing AAV genome and capsid engineering of an epigenetic-editing modality to develop a best-in-class treatment for Myotonic Dystrophy Type 1 (DM1)
- Day&Time : May 12th 5:00-6:30 PM EST

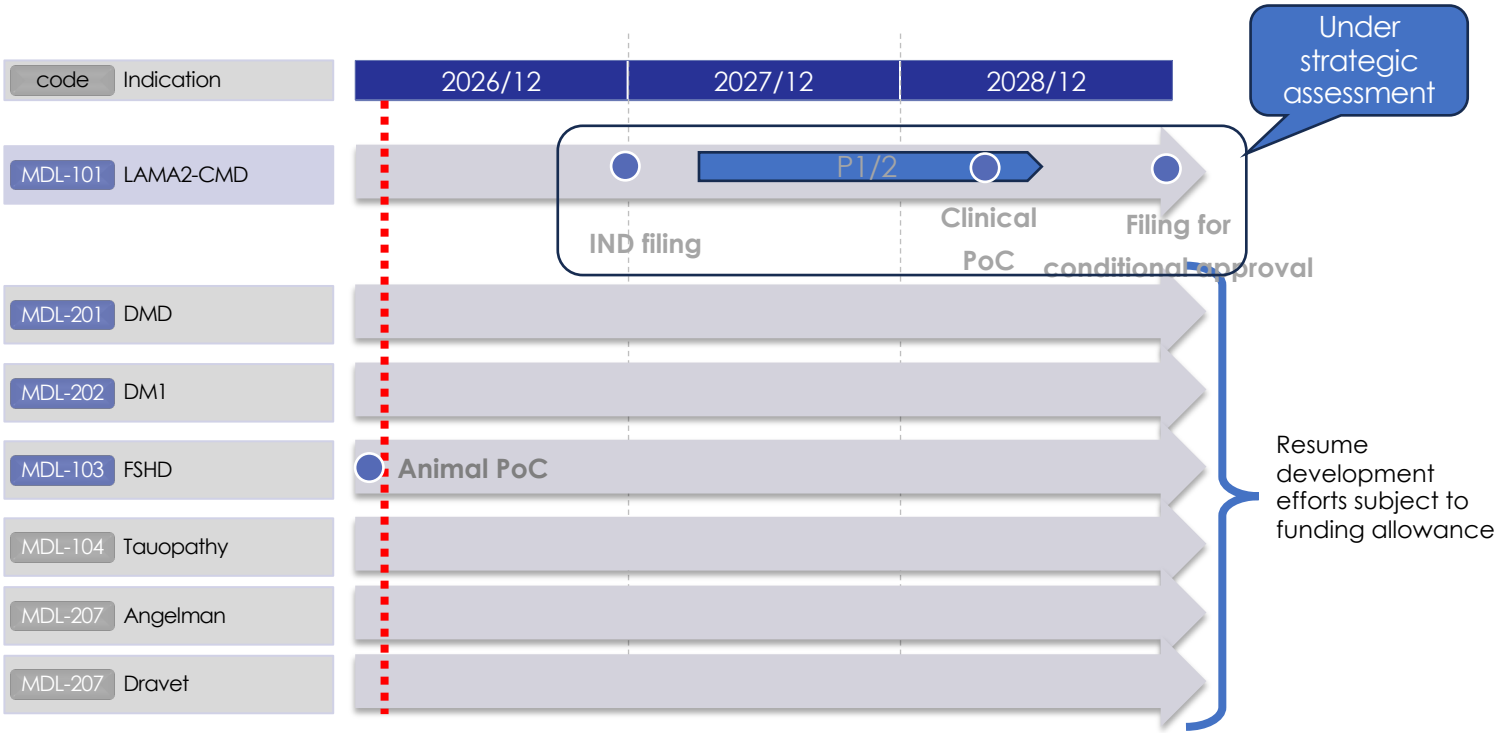
- Title: Epigenetic suppression of Dux4 using CRISPR-GNDM technology as a therapeutic for FSHD
- Day&Time : May 13th 5:00-6:30 PM EST

Updated

Pipeline status and coming milestones

Target filing for conditional approval in later 2028

Pipeline status



- Scheduled milestone events are informational in the future and subject to change

Updated

Achievements of the programs and coming milestones

	Achievement so far	Coming milestones
MDL-101 LAMA2-CMD	✓ Animal PoC ✓ Target engagement in NHP ✓ Pre-IND ✓ GMP manufacturing (Plasmid) ✓ ODD and RPDD designation ✓ Publication on HGT (Dec)	☐ Completion of Mice IND enabling(1H) ☐ **GLP-Tox** ☐ **GMP manuf.** ☐ **IND** ☐ **FPFD** } timing under strategic reassessment ☐ **Clinical PoC (2027~2028)** ☐ **Filing for conditional approval(2028)**

その他	• Established animal PoC
	  • MDL-201 (DMD)
	  • MDL-202 (DM1)
	  • MDL-103 (FSHD)
	  • MDL-104 (Tauopathy)
	  • MDL-205 (Angelman syndrome)
	  • MDL-207 (Dravet syndrome)
	• Research collaboration with JCR in CNS
	✓ **Data presentation at Solve FSHD conference and ASGCT (MDL-202, MDL-103)**
		• Explore optimal capsid and route of administration for CNS program
		• Allocation of development funds through partnering and grants
		• Animal PoC
		• Continuing Research and Moving to Next Steps

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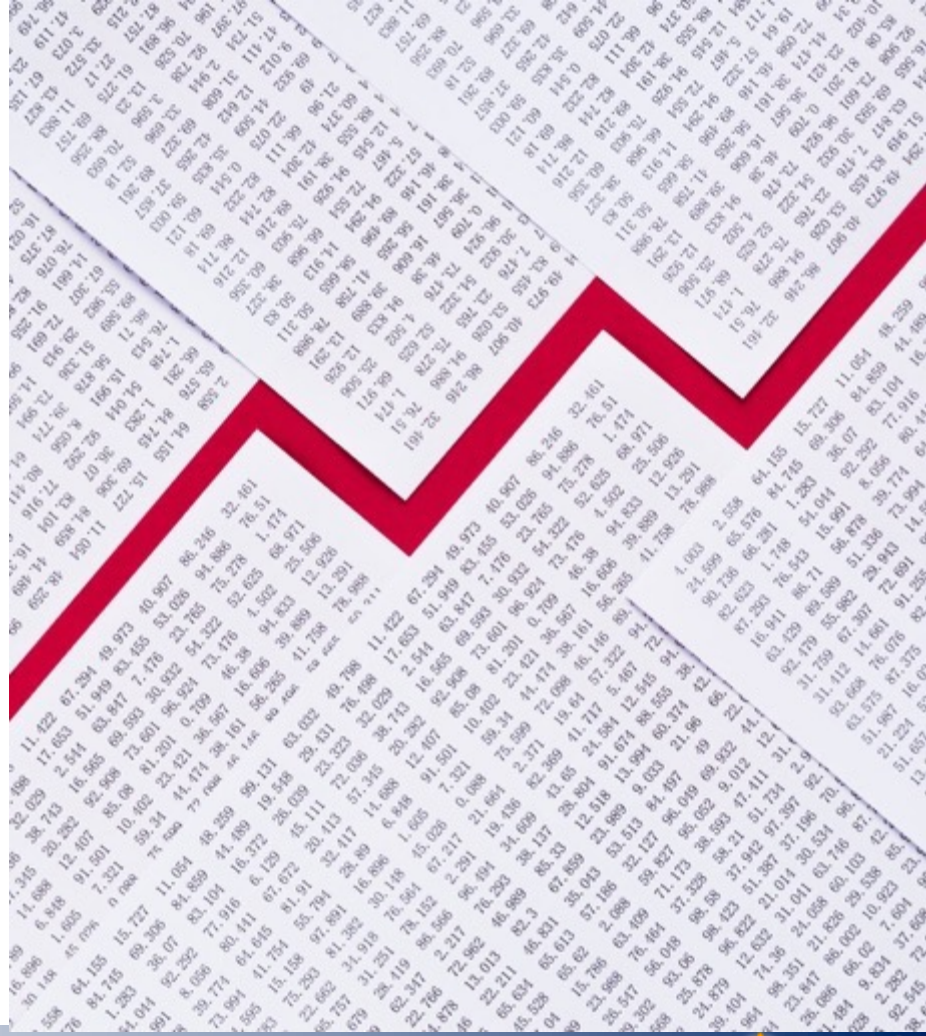
Progress on IP

UTRN patent was granted in South Korea

- Patent "METHOD FOR TREATING MUSCULAR DYSTROPHY BY TARGETING UTROPHIN GENE" related to **MDL-201** for DMD, granted in **South Korea** (Patent No. 10-2021-7018333) (Jan)

2. Financial updates at the end of FY2025



BS & Financial Position at the end of 1Q/2026

Maintain a certain level of cash and deposits needed for operations for ~12 months

(Million Yen)

	End of FY2025 (A)	1Q FY2026 (B)	(B) – (A)
Current assets	2,892	2,890	△2
Cash & deposits	2,812	2,812	0
Non-current assets	71	51	△20
Total assets	2,964	2,942	△22
Current liabilities	134	115	△19
Non-current liabilities	36	27	△9
Total liabilities	170	142	△28
Total net assets	2,793	2,799	6
Total liabilities and net assets	2,964	2,942	△22
Capital adequacy ratio	93.0%	93.8%	

Note

- While continuing investment in R&D, the exercise of stock acquisition rights progressed. As a result, cash and deposits decreased slightly.

Information purpose only

BS & Financial Position at the end of 1Q/2026

Maintain a certain level of cash and deposits needed for operations for ~12 months

(In thousand USD at @160yen/$)

	End of FY2025 (A)	1Q FY2026 (B)	(B) – (A)
Current assets	18,075	18,062	-13
Cash & deposits	17,575	17,575	0
Non-current assets	443	318	-125
Total assets	18,525	18,387	-138
Current liabilities	837	718	-119
Non-current liabilities	225	168	-57
Total liabilities	1,062	887	-175
Total net assets	17,456	17,493	37
Total liabilities and net assets	18,525	18,387	-138
Capital adequacy ratio	93.0%	93,8%	

Note

- While continuing investment in R&D, the exercise of stock acquisition rights progressed. As a result, cash and deposits decreased slightly.

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PL & Business Result at the end of 1Q/2026

342 million in operating expenses, mainly due to the cost of related to MDL-101 program

(Million Yen)

	1Q FY2025 (A)	1Q FY2026 (B)	(B)−(A)
Operating revenue	-	-	-
Operating expenses	632	342	△290
R&D	571	293	△278
SGA	61	48	△13
Operating income	△632	△342	290
Ordinary income	△651	△322	329
Current Profit	△652	△323	329

Operating expenses

- Continued investment in research and development as development activities centered on MDL-101 progressed.
- Meanwhile, continued promotion of financial strategies, including capital policy initiatives and the utilization of external financing.

PL & Business Result at the end of 1Q/2026

$2,1M in business expenses, mainly due to the cost of related to MDL-101 program

(In thousand USD at @160yen/$)

	1Q FY2025 (A)	1Q FY2026 (B)	(B)−(A)
Operating revenue	-	-	-
Operating expenses	3,950	2,137	-1813
R&D	3,568	1,831	-1737
SGA	381	300	-81
Operating income	-3,950	-2,137	1813
Ordinary income	-4,068	-2,012	2056
Current Profit	-4,075	-2,018	2057

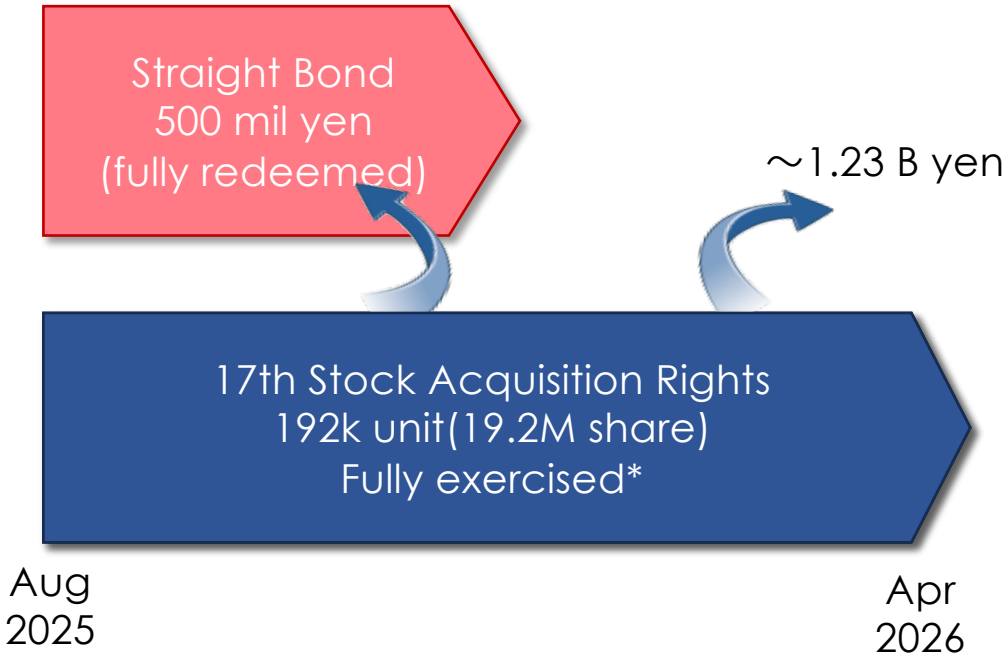
Operating expenses

- Continued investment in research and development as development activities centered on MDL-101 progressed.
- Meanwhile, continued promotion of financial strategies, including capital policy initiatives and the utilization of external financing.

Updated

The status of the 17th series of MS warrant with 1st series SB

The 17th series of warrant has been fully exercised



- Additional funding for MDL-101
- MDL-201 development costs
- R&D costs for MDL-103 and other subsequent pipeline products
- Administrative cost

*As of press release on Mar 17, 2026

3. Growth Strategy

Three-phase Growth Strategy

Diversified pipeline with their own missions

Pioneer the gene modulation With highly suitable indications

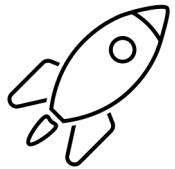
MDL-101

Expand technology opportunity with products for larger opportunity

MDL-201
MDL-202

Further approach to challenging applications

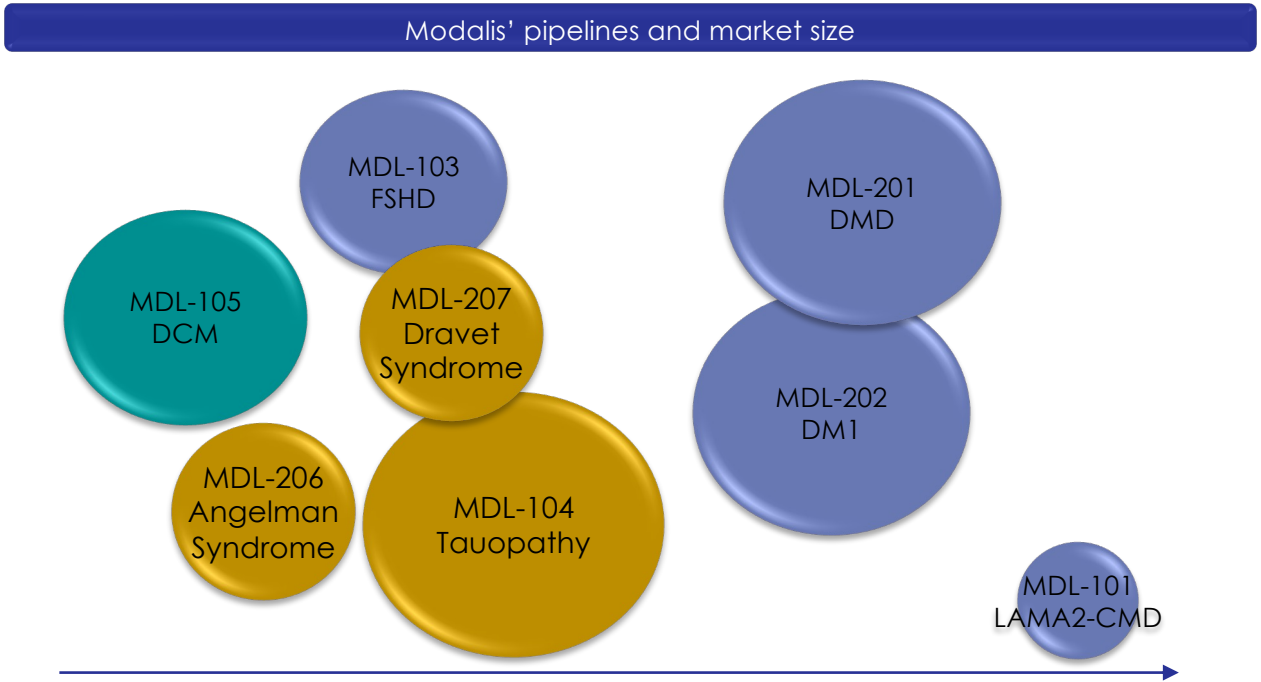
Other programs



Updated

Modalis' pipelines and market size

Large indication programs follow MDL-101 which paves the clinical path



※ Size of circles represents an image of market size or patient number of each indication

4. summary

Key Takeaway of 1Q/2026 report

1. Continuing preparations for the clinical transition of MDL-101. Confirmed improvements in long-term survival and functional outcomes in a disease model.

2. MDL-202 demonstrated superior DMPK inhibitory effects compared to the benchmark drug using a novel capsid.

3. MDL-103 demonstrated strong efficacy even at low doses in a disease model, and it was confirmed that it does not affect muscle regeneration.

4. DMD patent (MDL-201) granted in South Korea

5. Completion of the exercise of the 17th series of stock options (raised 1.23 billion yen)

Modalis Therapeutics

MODALIS

- 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

5. Q&A

Q1. Background of the Revision to the MDL-101 IND Timeline?

With regard to MDL-101, we are currently continuing to conduct GLP-tox studies and mouse IND-enabling studies, and to date, no significant new safety signals have been identified. Furthermore, we have not obtained any findings that would undermine the drug's efficacy or product concept, and we believe that the efficacy demonstrated in disease models and the results regarding LAMA1 expression induction continue to support this therapeutic concept.

Meanwhile, as MDL-101 is our top-priority pipeline candidate, we are optimizing our development plan—including CMC, the establishment of analytical systems, additional evaluations, and clinical trial preparations—to maximize the probability of success following the transition to clinical trials. In our discussions with clinicians, we have observed high unmet medical needs for LAMA2-CMD and significant expectations for this therapeutic approach; accordingly, we will continue to proceed with preparations for the clinical transition in a cautious and steady manner.

Q2. What are the key issues you are currently focusing on in preparation for clinical translation?

Our company is currently focusing its efforts, including additional analyses, primarily on the following areas:

- Advancement of analytical characterization
- Enhancement of the robustness of manufacturing and quality control processes
- Validation of long-term expression profiles
- Translational evaluation with a view to clinical application
- Optimization of dosage and administration strategies

Since MDL-101 is a pioneering development program for an epigenome-editing therapeutic using CRISPR-GNDM®, we believe it is important to conduct development with a view not only to an early IND application but also to maximizing product value after clinical transition and in the future.

Therefore, we have determined that thoroughly validating and optimizing these aspects at this stage is crucial for future regulatory approval, product competitiveness, and the enhancement of long-term business value, and we are proceeding with caution.

Q3. Given the ongoing review of the development plan, how do you view the value of MDL-101?

We believe that MDL-101 is a program with the potential to become a new treatment option for LAMA2-RD, a disease with significant unmet medical needs for which no effective treatments currently exist.

In particular, LAMA2-RD presents significant technical challenges for conventional gene replacement therapies due to factors such as gene size and tissue distribution; however, MDL-101 has the potential to address these challenges through its unique approach of inducing LAMA1.

Furthermore, as a systemically administered therapeutic agent utilizing gene activation via epigenome editing, MDL-101 is a program at the forefront globally, and we consider it a key pipeline demonstrating the clinical value of the entire CRISPR-GNDM® platform.

In non-clinical studies to date, results supporting this therapeutic concept have been obtained, including improved long-term survival and functional recovery in disease models, as well as target engagement in non-human primates. Based on these results, we believe it is important to proceed with development cautiously, with an eye toward maximizing long-term success rates and product value, rather than simply prioritizing an early transition to clinical trials.

We believe that the successful development of MDL-101 will not only offer new treatment possibilities for patients with LAMA2-RD but will also lead to the clinical validation of the novel therapeutic concept of epigenome editing via CRISPR-GNDM®, serving as a crucial foundation for future expansion into other diseases.

Q4. Given our current cash reserves, how long can we continue development?

Following the completion of the exercise of the 17th series of stock options, we have secured cash and deposit balances exceeding 2.8 billion yen as of the end of March 2026, thereby maintaining the financial foundation necessary to advance our ongoing major development activities.

In addition to raising funds from the capital markets, we are continuously exploring and promoting the diversification of funding sources, including grants, joint research, and partnering.

Our policy is to prioritize investment in key development programs, centered on MDL-101, while maintaining disciplined capital allocation, and to conduct business operations with the aim of maximizing corporate value over the medium to long term.

Q5. How does company view the competitive advantages of the MDL-202?

The data disclosed in this announcement show that MDL-202 demonstrated strong DMPK inhibitory effects in disease models compared to previously reported benchmark drugs. In particular, we consider the observation of high inhibitory effects in the myocardium and diaphragm—key disease-relevant tissues in DM1—to be a key feature of this program.

Furthermore, since MDL-202 does not repair the DMPK mutation itself but rather suppresses the expression of pathogenic RNA through transcriptional regulation, it has the potential to be applicable to a broad range of patients, independent of specific mutation types.

We believe these results were achieved through the synergistic effects of combining the novel muscle-targeted capsid with the CRISPR-GNDM® molecule.

Furthermore, data supporting the efficacy of CRISPR-GNDM® technology has also been obtained in other muscular disease programs, such as MDL-103 for FSHD and MDL-201 for DMD. We plan to continue the development of each program while proceeding with evaluations, including those regarding durability and translational assessment.

Q6. Competition in the epigenome editing field is intensifying. What sets Modalis apart?

In recent years, there has been an increase in new entrants to the field of epigenome editing using CRISPR technology, with numerous programs being developed, particularly those focused on gene silencing. At the same time, we believe that for modalities such as gene therapy—which involve high technical hurdles and inherent risks—to gain widespread acceptance in the future, it is essential to first demonstrate their value in disease areas with significant unmet medical needs where existing treatments fall short.

From this perspective, we have adopted a unique approach of gene activation via epigenome editing for diseases—such as LAMA2-RD—where conventional gene replacement therapies face significant technical constraints. We believe that the field of muscular disorders, in particular, requires advanced technical capabilities—including systemic delivery, sustained expression, and tissue selectivity—while also representing an area with immense unmet medical needs.

Furthermore, while development in the current epigenome editing field is predominantly centered on gene silencing, we are one of the few companies focused on gene activation, and we consider this to be a key differentiator.

We believe that by combining our expertise in CRISPR-GNDM®-based gene activation technology, muscle-targeted delivery technology, and the development of systemically administered AAVs, we will continue to maintain a leading position in this field.