English translation for reference purposes only

Non-cleaving CRISPR as A Novel Therapeutic Modality for Genetic Disorders

The switch  is the Key

(TSE4883) Modalis therapeutics Corporation

Corporate and Technology summary

08. 2020

In case of any discrepancy, the Japanese version shall prevail

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Corporate Overview

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Corporate Overview

Modalis Therapeutics is a biotech company to develop CRISPR-based gene therapies

Name	Modalis Therapeutics Corporation
Foundation	Jan 2016
President CEO	Haru Morita
HQs	16-5 Nihonbashi-Kabuto-cho, Chuo—ku, Tokyo 103-0026 Japan
US subsidiary	Modalis Therapeutics Inc. (51 Moulton st. Cambridge MA)
Business	Drug Development
Common stock	13,000,000 thousand yen
Capital Raised	41.1Billion Yen (~38.4 Million USD)
Outstanding share	25,100,000 common stock (excluding 2,903,200 stock option units)
# of employee	17 (including 7 PhD) (4 in Japan, 13 in US) As of end May 2020
Award	J-STARTUP (2018)

Date	History
Jan 2016	Founded in Tokyo as EdiGENE Corporation
Apr 2016	Established 100% owned US subsidiary EdiGENE Inc. (Now Modalis Therapeutics Inc.)
Apr 2017	Entered into research collaboration with **Astellas** Pharma Inc.
Dec 2017	Series A round from **FUJIFILM** Corporation and 8 other companies through third-party allotment
Dec 2017	Expanded research collaboration with **Astellas**
Jun 2018	Selected as a **J-Startup** program, an elite program funded by the Japanese government.
Jan 2019	HQs moved to new facility
Mar 2019	Established license agreement on a genetic disorder with **Astellas** Pharma Inc.
Mar 2019	Cambridge Lab moved to the new facility
Aug 2019	Company name changed to Modalis Therapeutics
Sep 2019	Established 2nd license agreement on a genetic disorder with **Astellas** Pharma Inc.
Nov 2019	Entered into research collaboration with **Eisai** Inc.
Apr 2020	Entered into a license agreement with **Editas Medicine**, Inc to obtain access to foundational CRISPR IP.

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Modalis' 6 strengths

In the right time, the right place, with the powerful technology and the best brightest

GTx and Gene Editing, the "next big thing"

- **Expected Strong market growth** after the dawn period
- Huge opportunities of **100s monogenic disorders**

Leading non-cleaving CRISPR company

- The **precision** of gene editing but even **safer** as it doesn't cut DNA
- **unique IP position** with access to CRISPR foundational IP

Diversified and scalable pipelines

- Solid discovery process and predictable clinical outcome
- **5** on-going collaboration pipelines with large pharma companies and **2** internal pipelines

Pharma Partnerships

- Collaboration with **3** multinational pharma companies
- Total milestone of the 2 out license programs worth **>$350M**

Hybrid pipeline model

- Hybrid of earlier cash flow from **collaboration pipeline** and upside potential from **Internal pipeline**
- Came to **ordinary profits positive** in FY2019

Strong team

- Combination of seasoned management and talented scientists from all over the world

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Management Team and Board of Directors
Seasoned team

Co-founder, President, CEO, and Chair of the board
Haru Morita
- REGiMMUNE, Founder, President and CEO
- Y's Therapeutics, Booz Allen and Company, Kyowa-KIRIN

Board member, founding scientist
Osamu Nureki Ph.D
- Professor, Department of Biological Sciences, Graduate School of Science, The University of Tokyo
- Purple Ribbon medal (Shiju-Hou-shou)

SVP, Chief Technology Officer
Tetsuya Yamagata, M.D. Ph.D
- Glaxo Smith Kline, Tempero Pharmaceuticals
- Joslin Diabetes Center, Harvard Medical School

Board member
Hideki Takeda
- President, Medical Patent Research
- Healios KK former president, Fujisawa (merged into Astellas)

VP, Chief Financial Officer
Naoki Kobayashi, MBA
- Former CFO at Oncolysbiopharma, Hatena, and Argens
- Deloitte Tohmatsu, Daikyo Real estate

Board member
Joseph S. McCracken, DVM
- Roche Head of Global license, Genentech, Sanofi

Board member, Audit committee
Miyuki Shimane
- Chugai Pharma

Board member, Audit committee, CPA
Teruhisa Tajima
- President Tajima CPA office
- Corporate Auditor (Quantum Biosystems, OncoTherapy, Prism Bioscience)

Board member, Audit committee, Attorney at Law
Toshio Furuta
- Representing attorney, Clair Law Firm
- Corporate Auditor (NetYear Group, Canbas, Zenrin)

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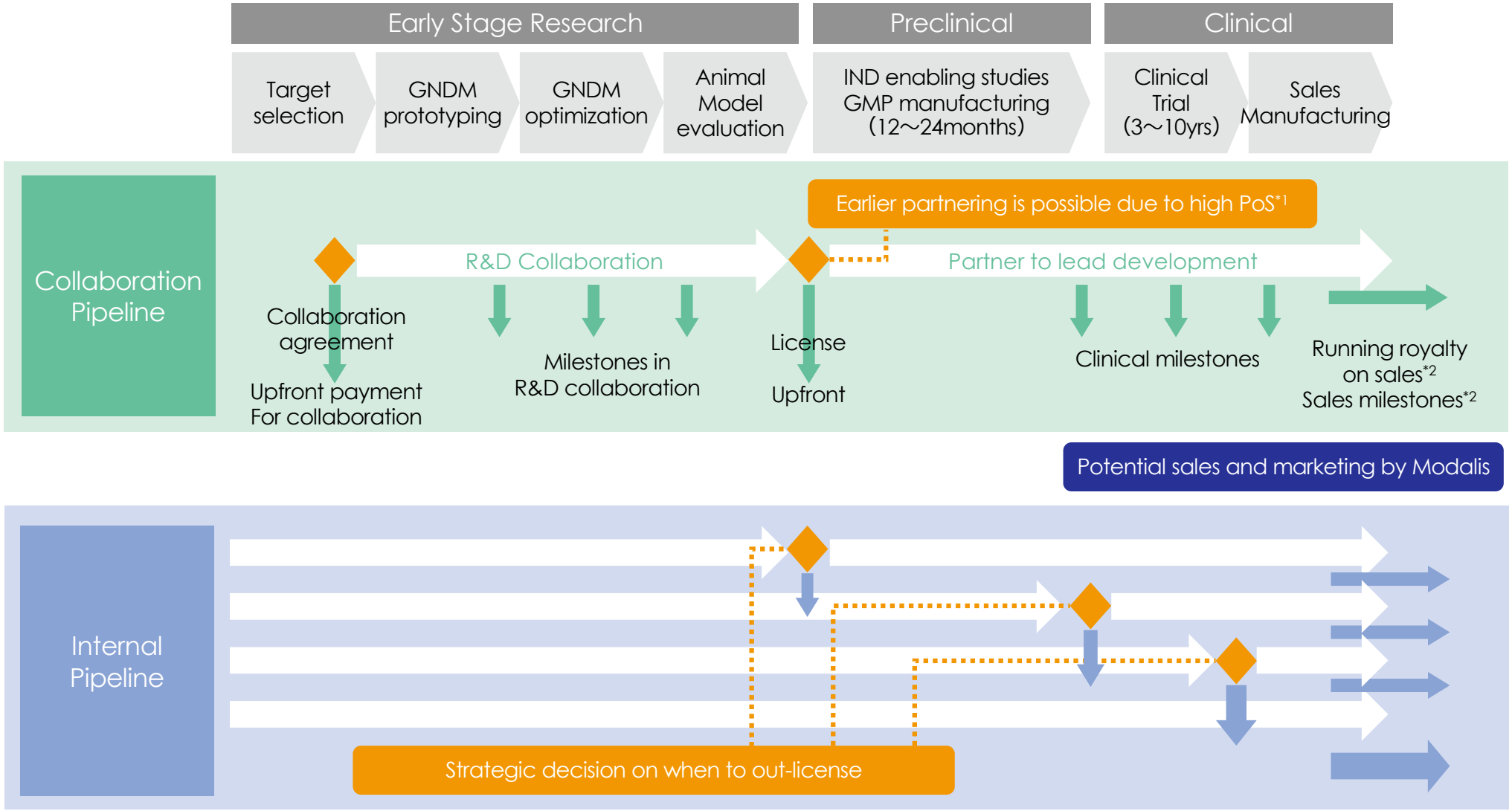
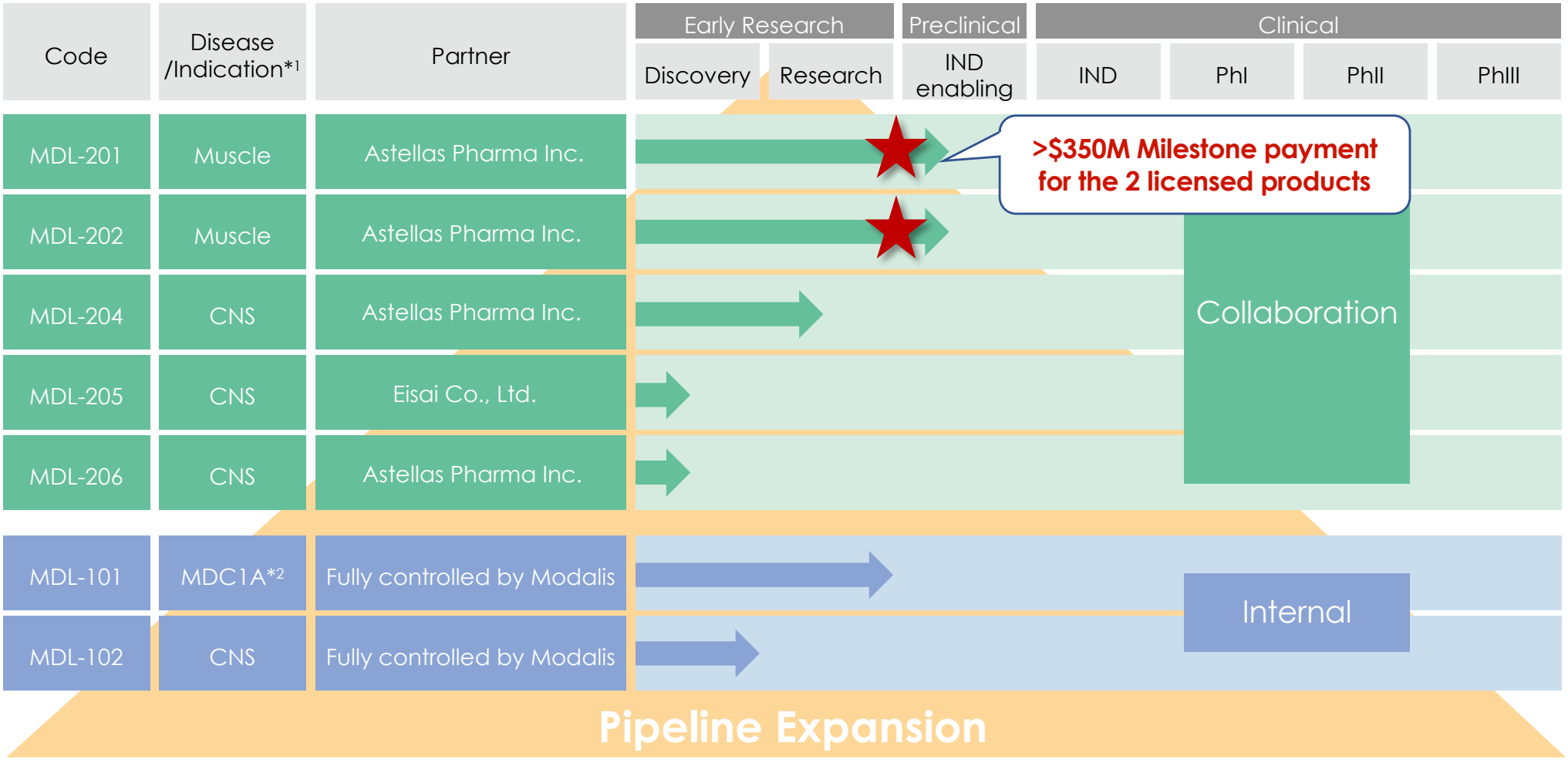
Business Model

Hybrid of own pipeline and collaboration pipeline



*1: detailed in P21
*2: future plan

Scalable pipeline



*1: We have adopted a strategy of withholding specific indications until the patent application is published for competitive reasons.
*2: MDC1A=Merosin-deficient congenital muscular dystrophy type 1A

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Financial Highlights

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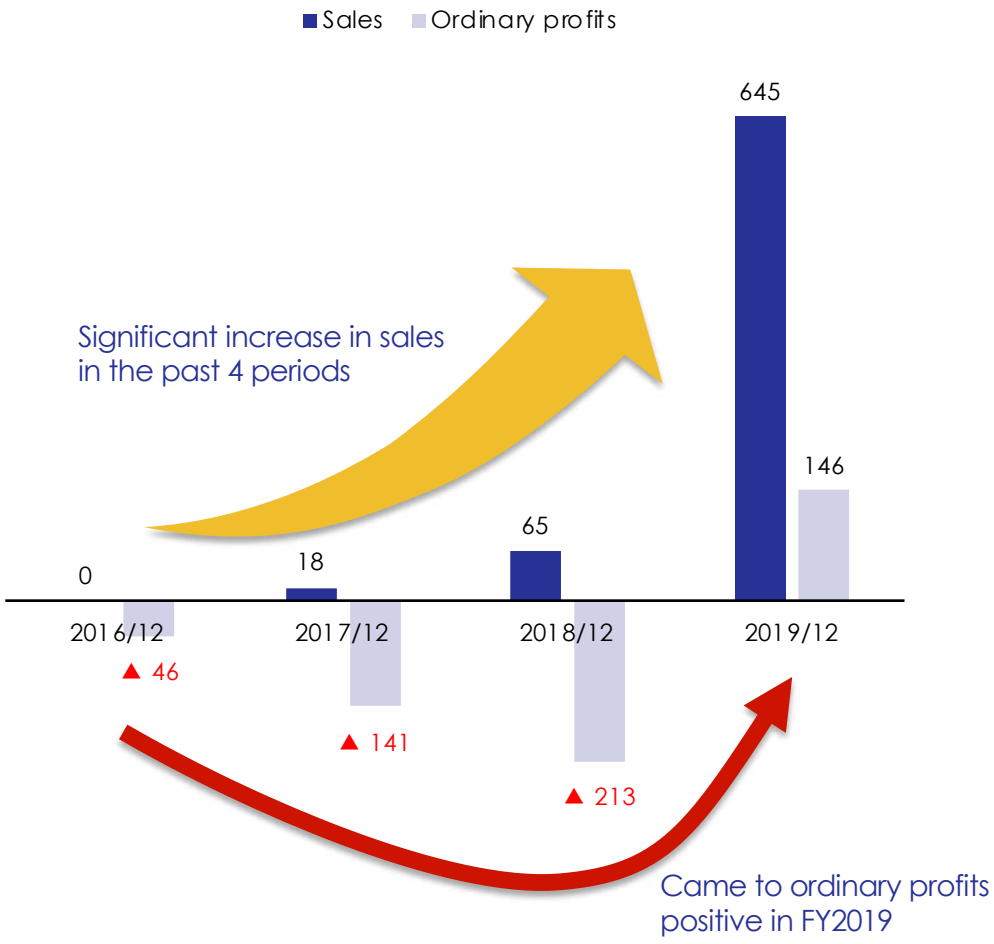
Sales, Profits, R&D expense and FTEs

With license deals, ordinary profits have become positive at the end of 2019

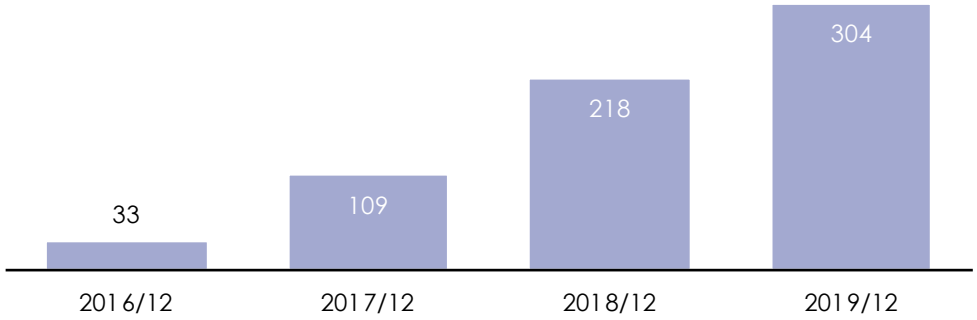
Sales and Profits

(Million Yen)

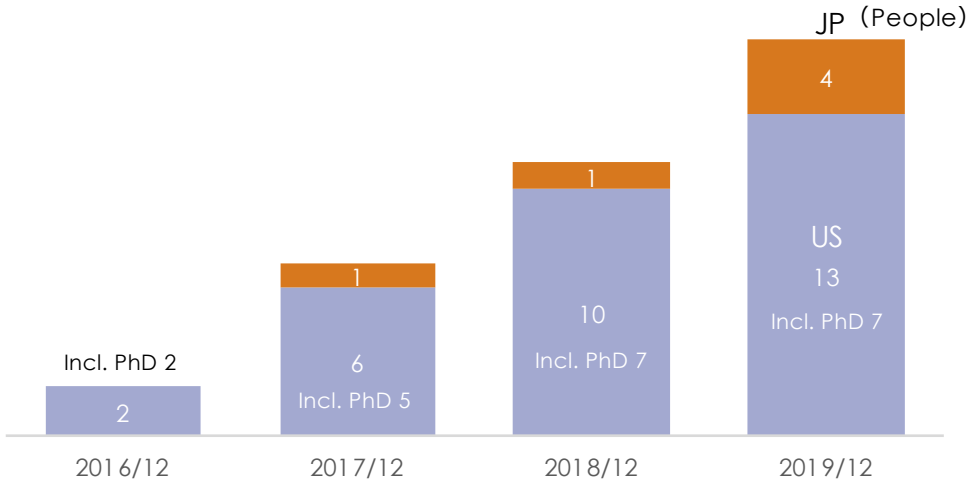


R&D expense

(Million Yen)



Number of FTEs (excluding board members



* 2016 – 2017 are non-consolidated basis, after 2018 are consolidated basis

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Financial Highlights

Stable financial base & shareholder composition

Consolidated B/S *1

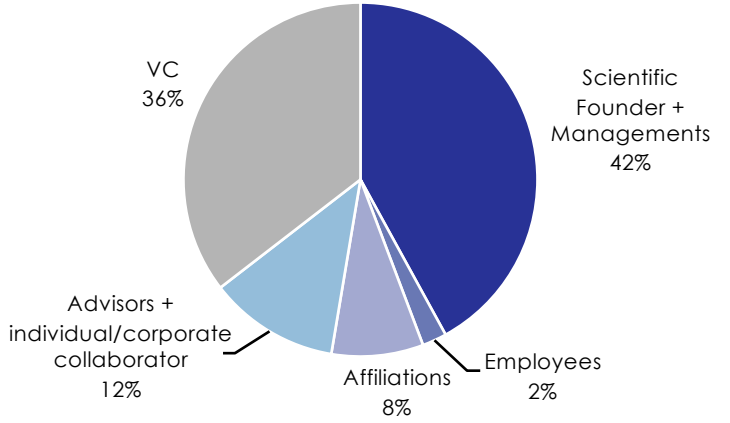
(Thousand Yen)	2018/12	2019/12	2020/12 1Q
Total current assets	1,214,738	3,874,974	3,703,938
Cash and cash equivalents	1,205,143	3,857,235	3,682,880
Total intangible assets	757	48,954	51,763
Investments and other assets	9,011	14,499	14,199
Total assets	1,224,508	3,938,428	3,769,901
Total Liabilities	22,728	95,885	45,446
Common stock	50,000	1,300,000	1,300,000
Capital surplus	1,371,735	2,621,735	2,621,735
Retained earnings (Accumulated deficit)	▲ 219,640	▲ 79,112	▲ 197,398
Total shareholders' equity	1,202,094	3,842,623	3,724,336
Total net assets	1,201,779	3,842,542	3,724,455
TOTAL LIABILITIES AND NET ASSETS	1,224,508	3,938,428	3,769,901

Consolidated P/L *1

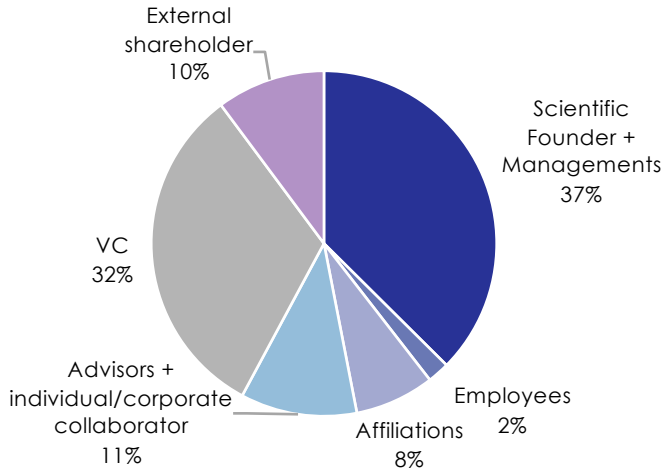
(Thousand Yen)	2018/12	2019/12	2020/12 1Q
License revenue	65,297	644,500	13,000
Total operating expenses	279,420	487,305	129,164
Research and development	217,927	303,680	81,317
Selling, general and administrative expenses	61,493	183,625	47,846
Operating profits (or loss)	▲ 214,123	157,194	▲ 116,164
Non-operating income	983	1,119	98
Non-operating expenses	250	11,962	858
Ordinary profits (or loss)	▲ 213,390	146,351	▲ 116,924
Profits (or Loss) before income taxes	▲ 213,390	146,351	▲ 116,924
Total income taxes	4,518	5,823	1,362
Net profits (or loss)	▲ 217,909	140,528	▲ 118,286
Profits (or Loss) attributable to owners of parent	▲ 217,909	140,528	▲ 118,286

*1: This is a translation of the original release in Japanese. In the event of any discrepancy, the original release in Japanese shall prevail.

Shareholder composition Before IPO (Incl. Potential stocks)



Shareholder composition After IPO (Incl. Potential stocks) *2



*2: Assumption after conducting public offering, sales, OA (upper limit) described in the prospectus

Our Features and Strong points

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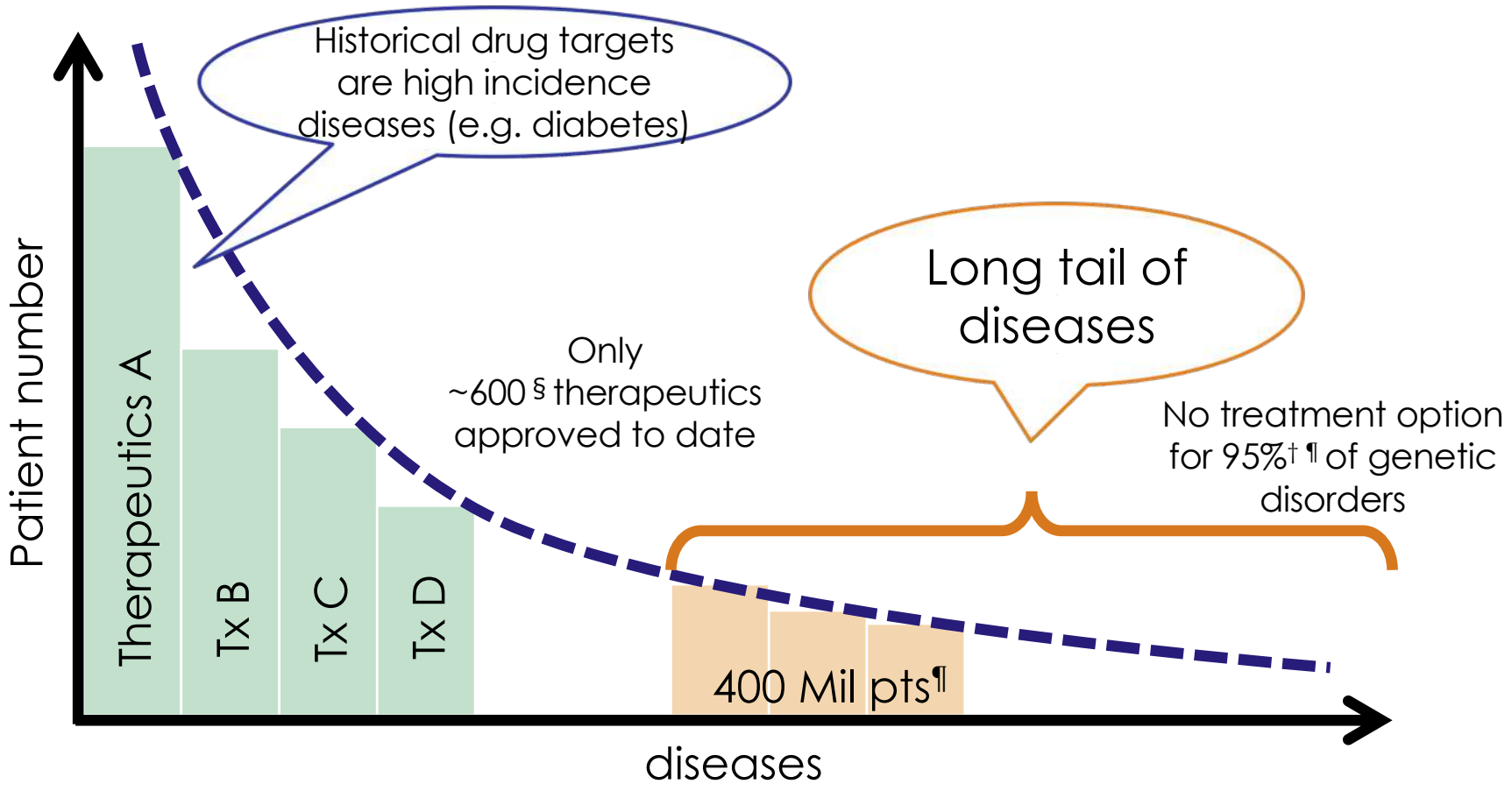
To target "long tail" of diseases, innovation is needed

Among 10,000* human disorders ~7,000#are orphan disease and 80%†are genetic disorders



Scalable efficient approach is required to tackle the divided population

Source: *21st Century Cure Act, #NIH GARD †innovation.org ¶GlobalGenes.org
§Active therapeutics of 491 NME, 106 BLA, 17 cellular and gene therapy products @FDA as of 2019.7.22 Source from KEGG

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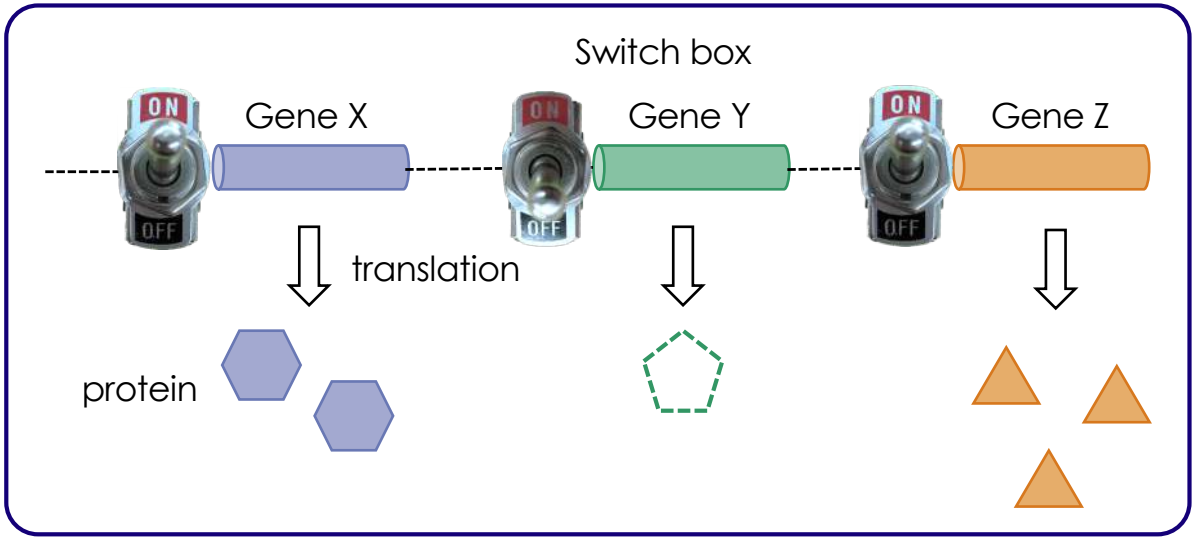
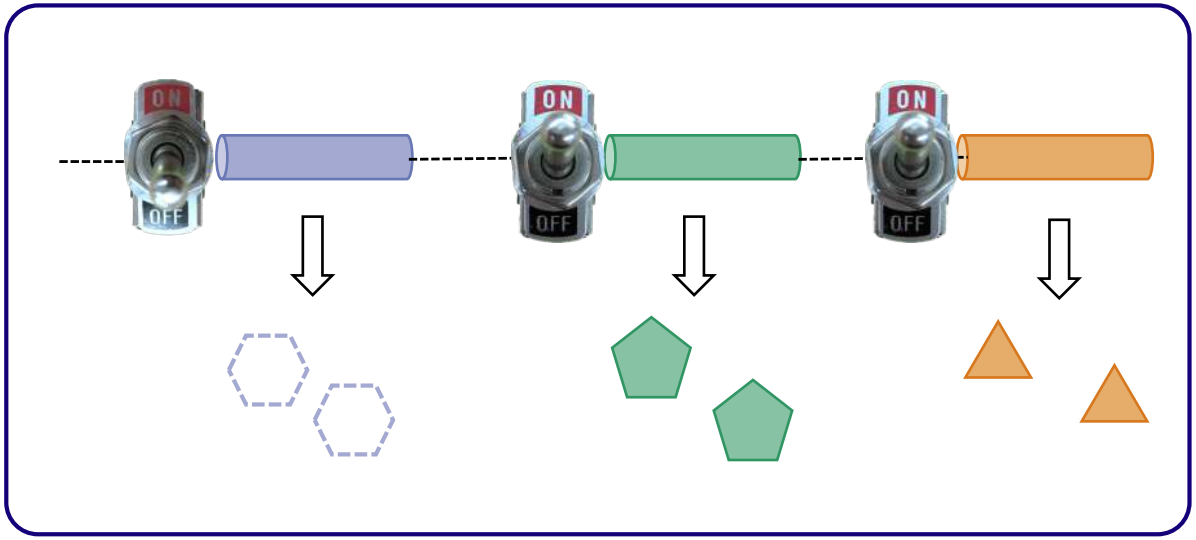
How multiple cell types are created from the same DNA code

Each of 20,000 genes have ON/OFF switches that control cell type specific expression

Cell A



Cell B



- There are **37.2 Trillion cells** in our body
- **200 cell types** in our body have the same DNA code despite differences in appearance and function
- Differences in cell types and their states are controlled by ON / OFF switches of the expression of **20,000 genes** coded by **3 billion bases of DNA**

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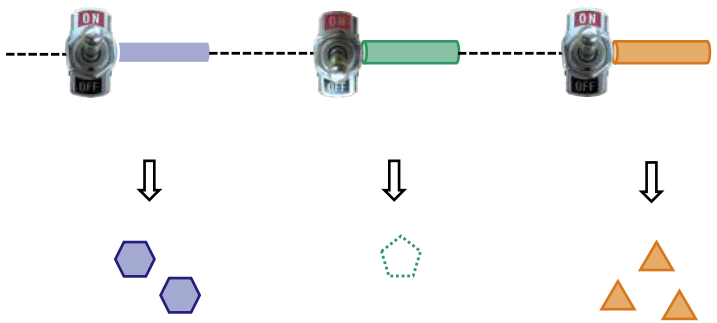
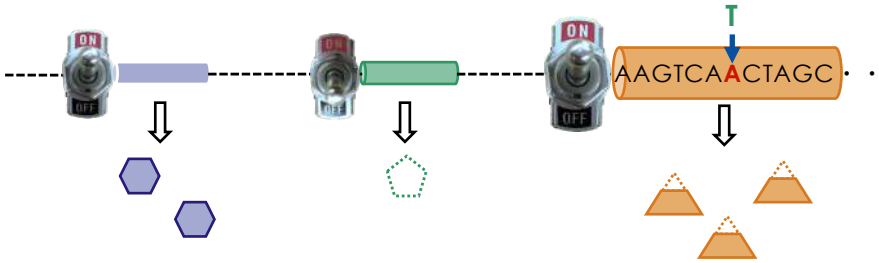
3 types of genetic disorders

An error in the code or in switching can cause disorders

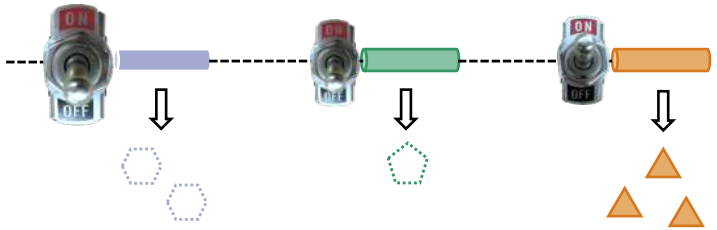
Normal state



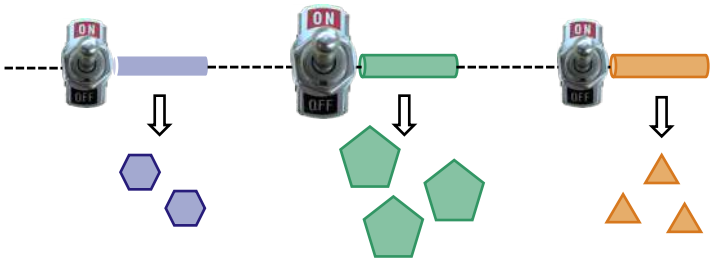
Error in the code results in a **non-functional protein**



A **required protein** is not supplied due to being witched OFF due to a switching error



An **un-necessary protein** is produced by a switching error



Switches control which, when and how much protein is expressed

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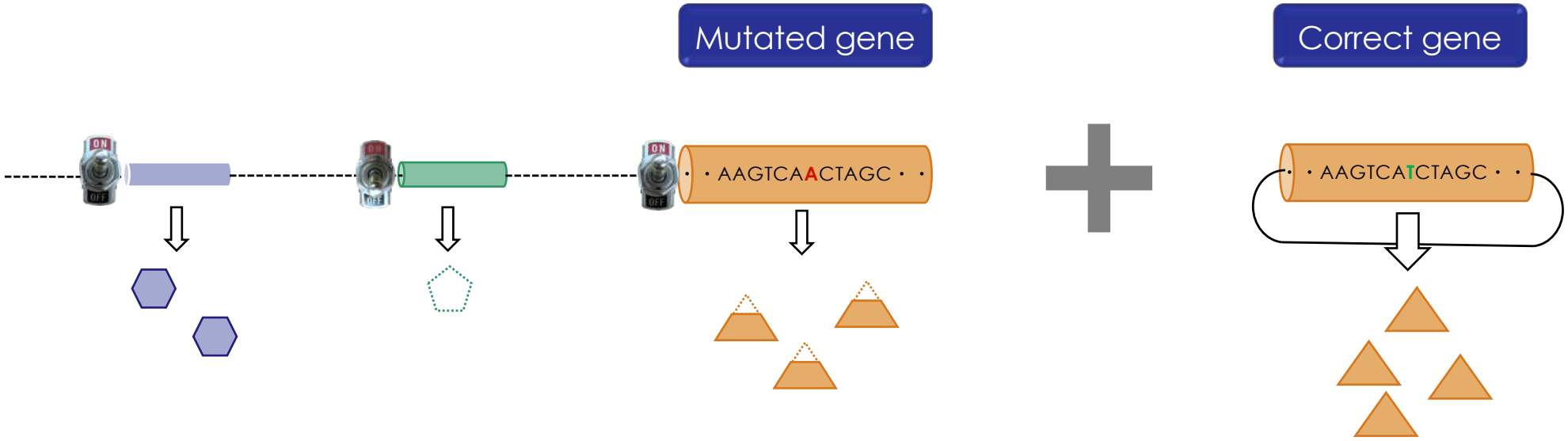
Classical gene therapy

Replaces a mutated gene by inserting a correct gene exogenously



Overwrite of missing gene

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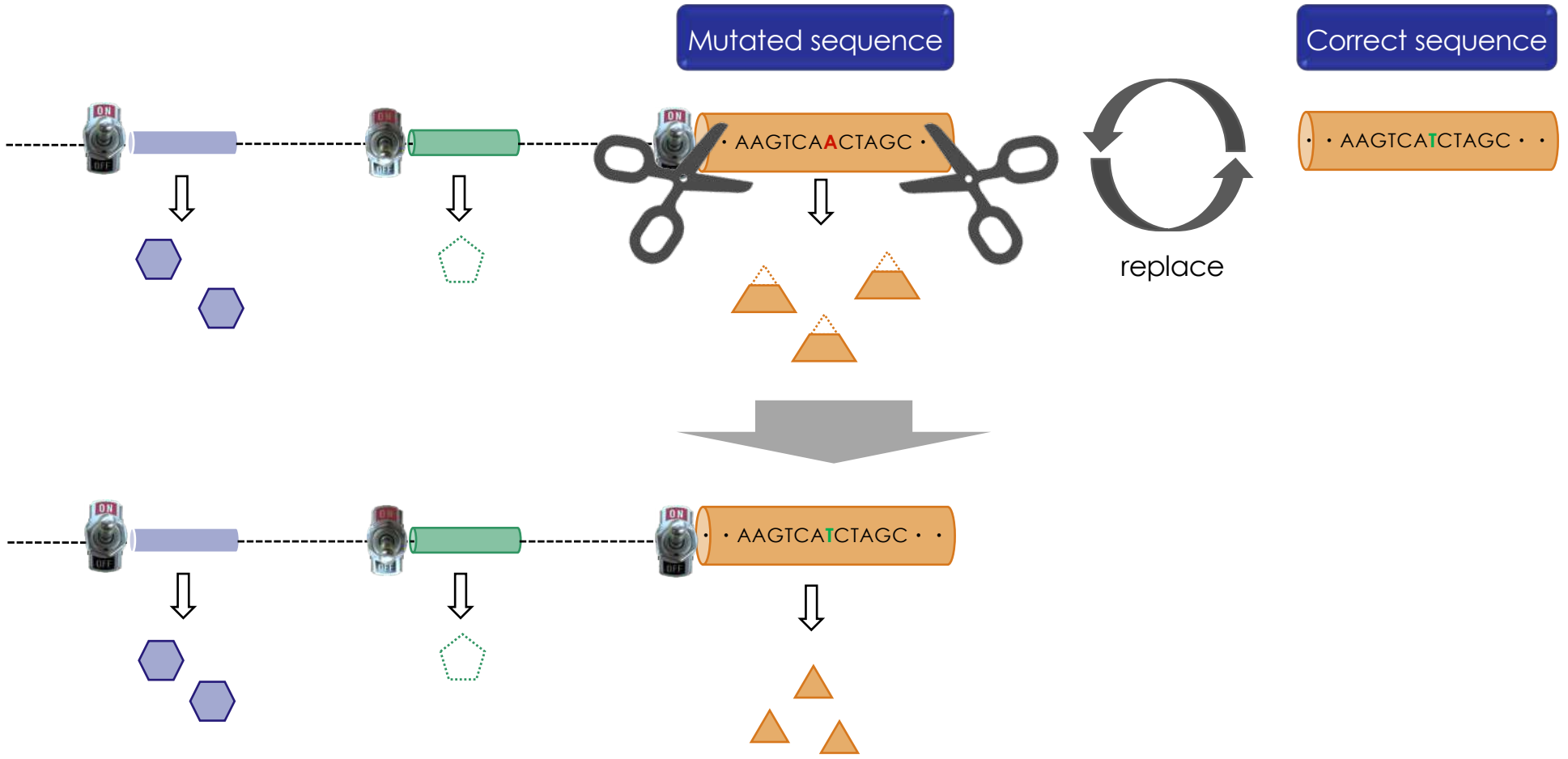
Gene Editing

Replaces mutated DNA with correct DNA by cleaving DNA



However, cleaving DNA increases risk of cancer

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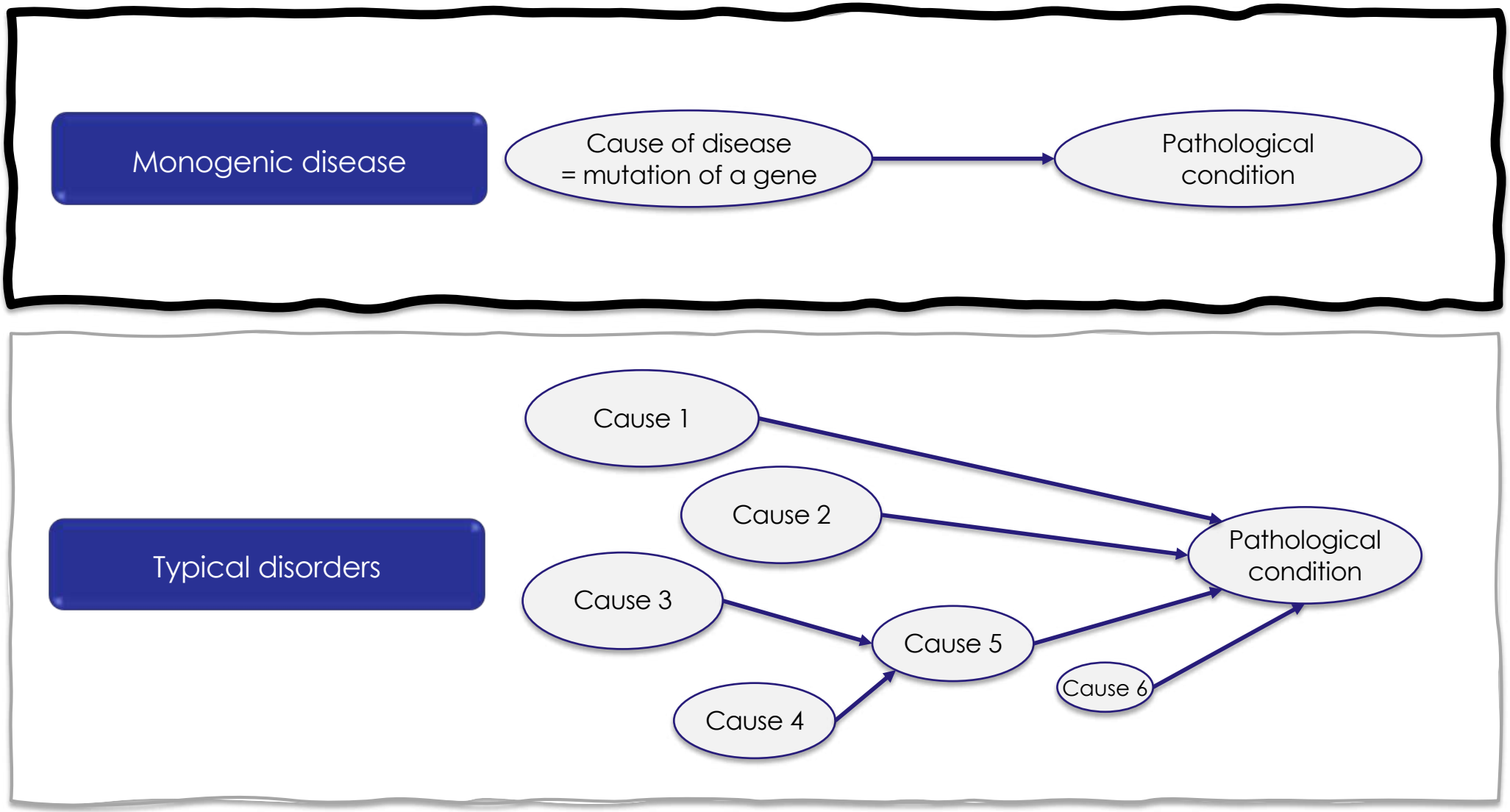
Monogenic Diseases

Gene therapies for monogenic disorders are expected to have higher probability of success due to simplicity of mechanism



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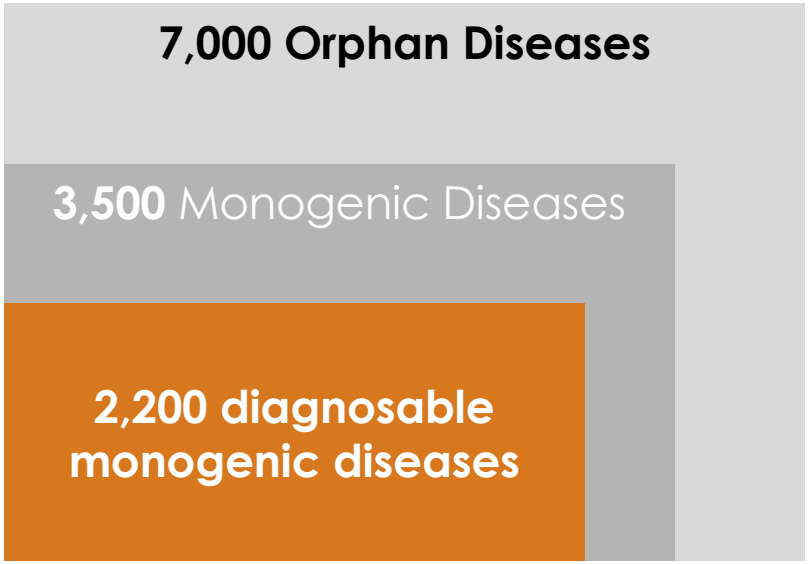
Untapped opportunities in monogenic disorders

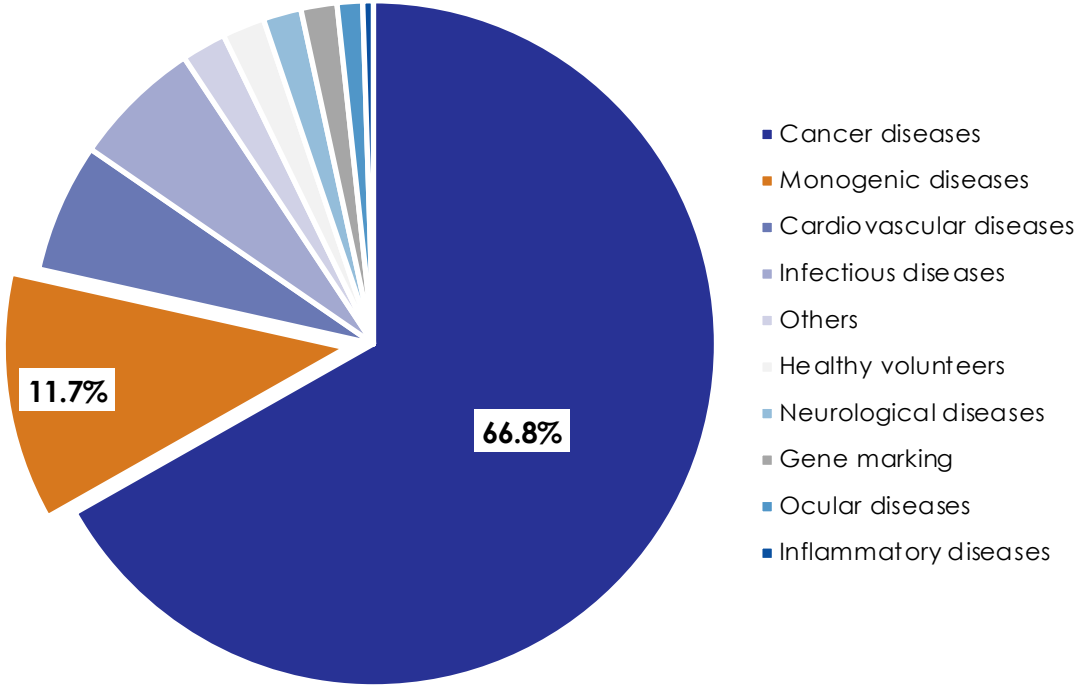
Limited number of drugs are approved or in clinical development for monogenic disorders

Monogenic Disease



Source: Discovery Medicine

Breakdown of GTx clinical trials (1989~ Worldwide)



Source: The Journal of Gene Medicine (2019)

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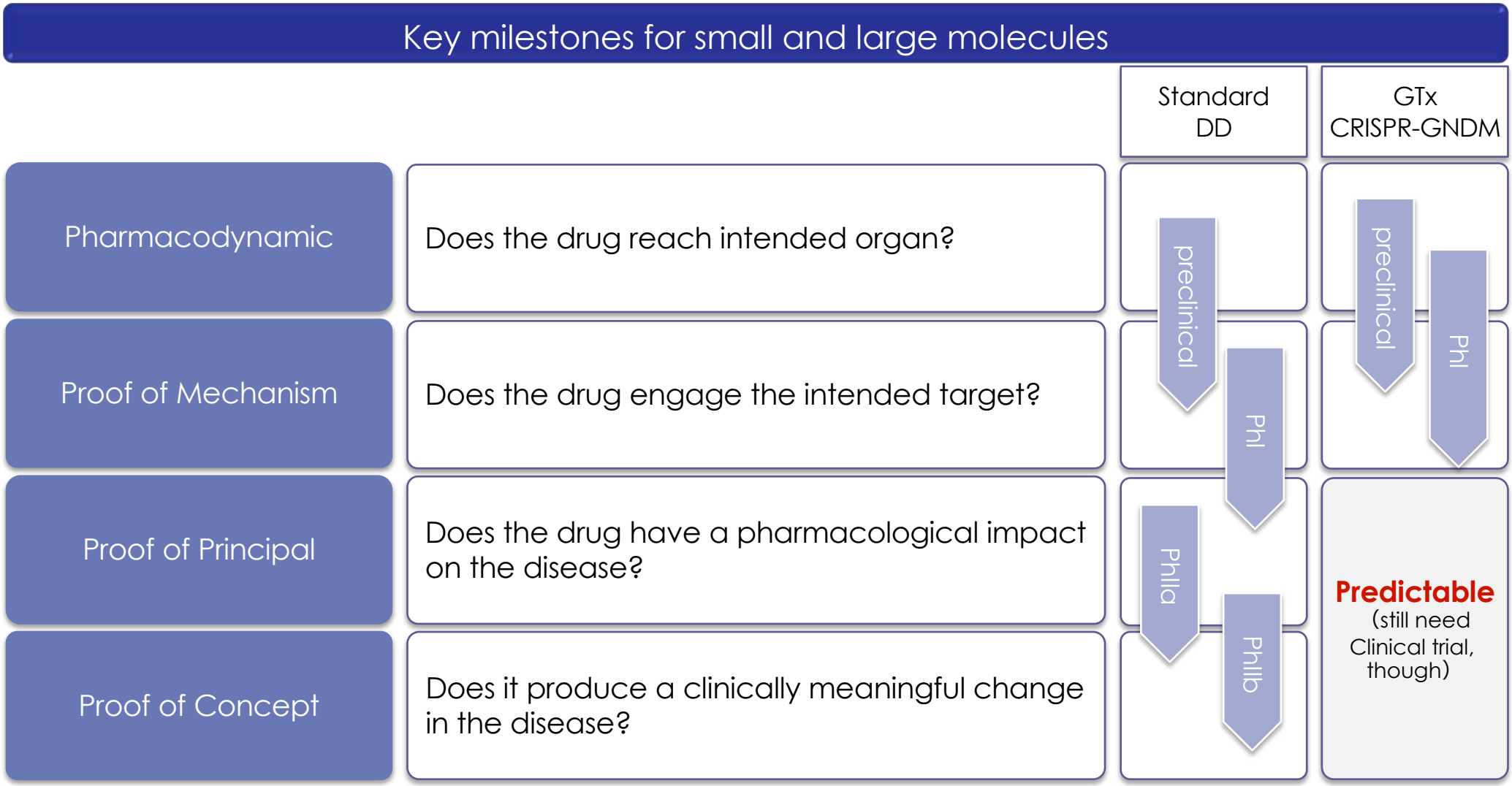
Major hurdles in drug development

PoP and PoC are predictable in GNDM prior to human testing



Source : Nature Biotechnology volume 30, p596–599 (2012)

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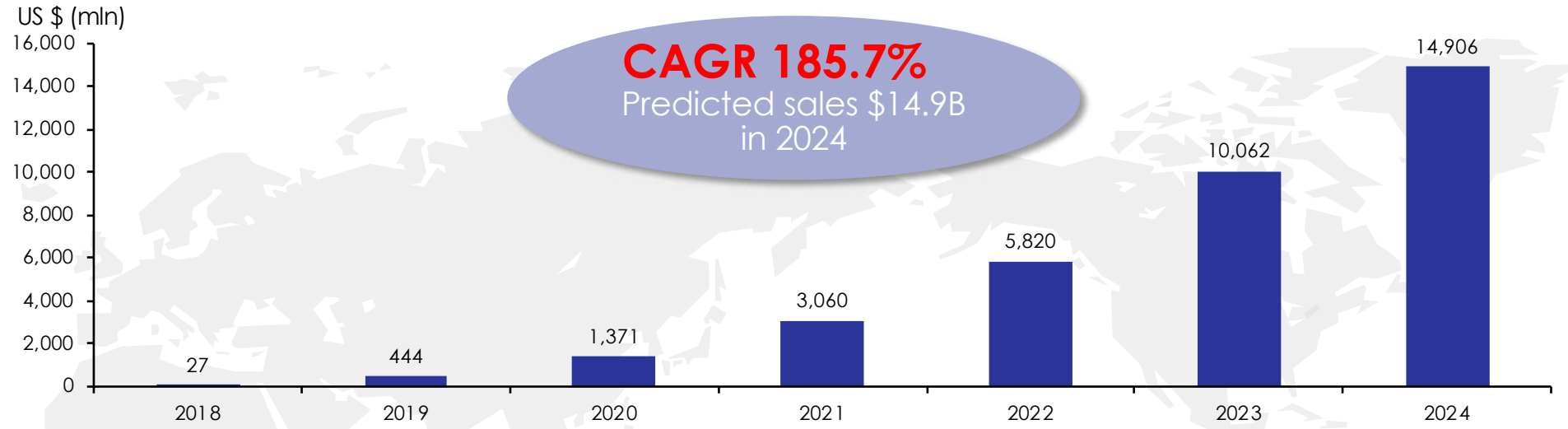
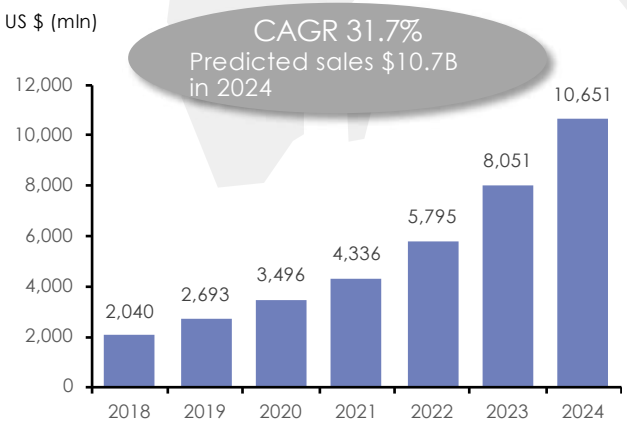
Sales growth of pharmaceutical modalities

GTx is growing faster than other modalities

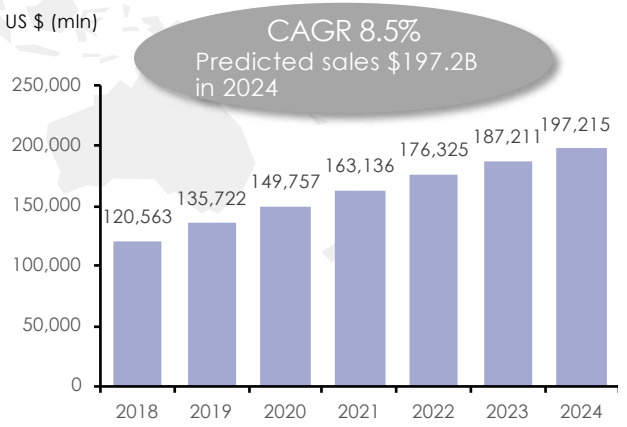
Predicted sales of Gene Therapy (WW)



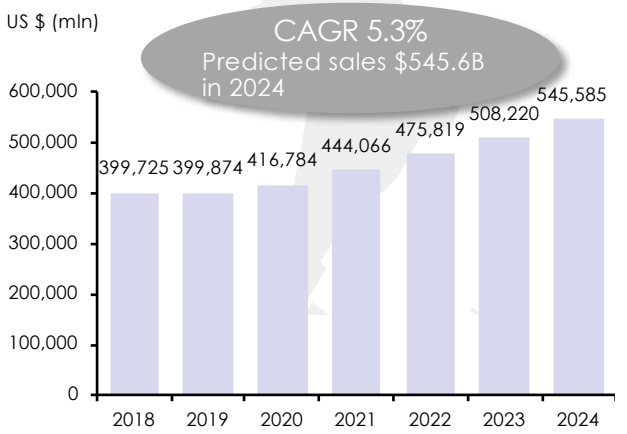
DNA&RNA therapeutics



Monoclonal antibody



Small molecule



Source : Evaluate Ltd (in Aug 2019 data) *CAGR=2018 to 2024. 2019 to 2024 are predicted sales

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Examples of GTx companies M&A

Acquisitions / investments in 2018-2019 deals ranged from millions to billions of US dollars

Name	Closing Date	Acquisition / investment	Deal total	Ref. (disclosed information by each company of GTx)
Astellas	2019/12	**AUDENTES Tx**	**$3.0 billion**	AT132: X-linked myotubular myopathy (XLMTM) AT845: Pompe disease AT702, AT751, AT753: Duchenne muscular dystrophy (DMD)
VERTEX	2019/6	**Exonics Tx**	**$1.0 billion**	Duchenne muscular dystrophy (DMD) myotonic dystrophy type 1 (DM1)
SAREPTA Tx	2019/3	**Myonexus Tx**	**$165 million**	MYO-101: limb-girdle muscular dystrophy 2E (LGMD 2E) MYO-102: limb-girdle muscular dystrophy 2D (LGMD 2D)
Biogen	2019/3	**Nightstar Tx**	**$800 million**	NSR-REP1: Choroid atrophy (Choroideremia) NSR-RPGR: X-linked retinitis pigmentosa (XLRP) NSR-ABCA4: Staggered disease
Roche	2019/2	**Spark Tx**	**$4.3 billion**	SPK-9001: Hemophilia B SPK-8011, SPK-8016: Hemophilia A SPK-7001: Choroid atrophy (Choroideremia)
NOVARTIS	2018/5	**AveXis**	**$8.7 billion**	Zorgensma AVXS-101（IT）: Spinal muscular atrophy type II（SMA Type 2） AVXS-201: Rett syndrome（RTT） AVXS-301: Amyotrophic lateral sclerosis（ALS）

Source : disclosed information by each company

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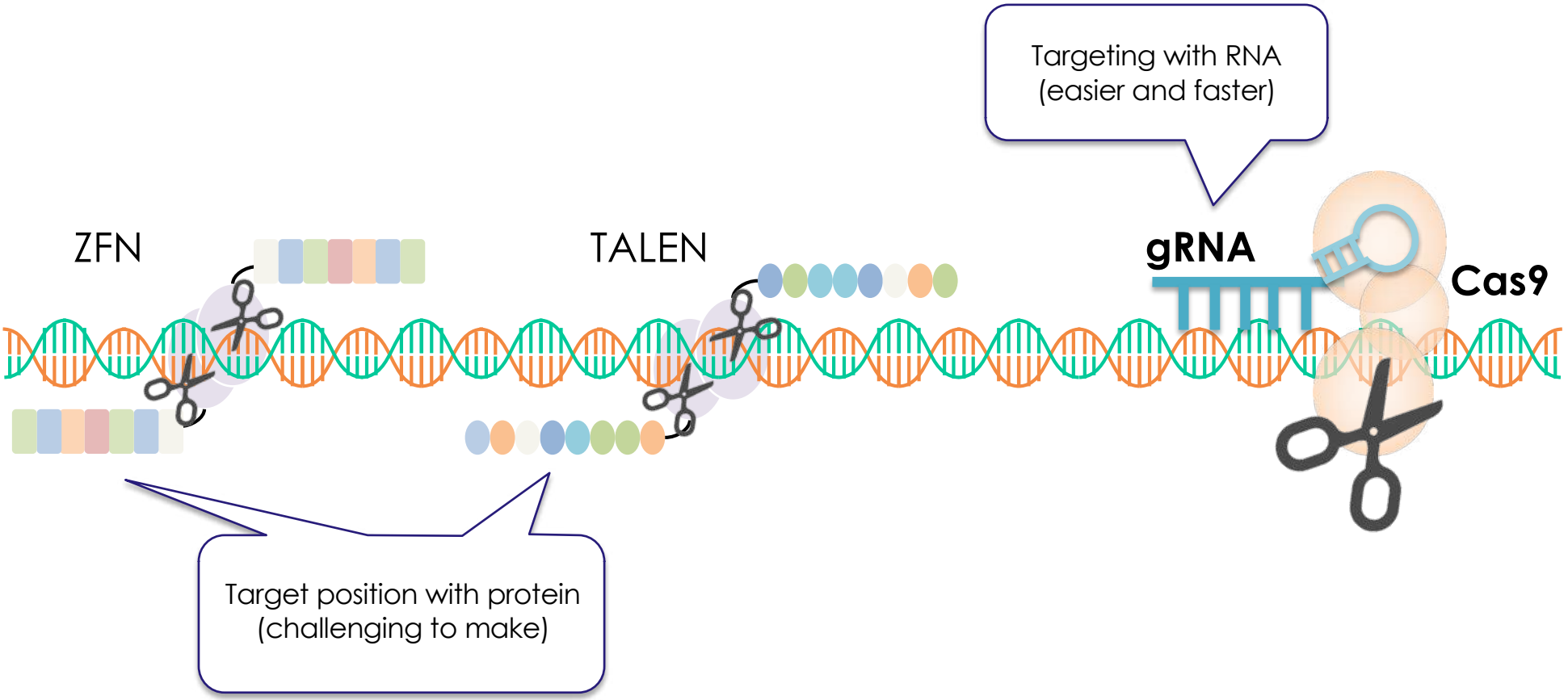
CRISPR is a novel gene editing technology

Faster and higher throughput due to limited variable region which is easily synthesizable as gRNA

Old gene editing technologies

- ZFN
- TALEN

CRISPR



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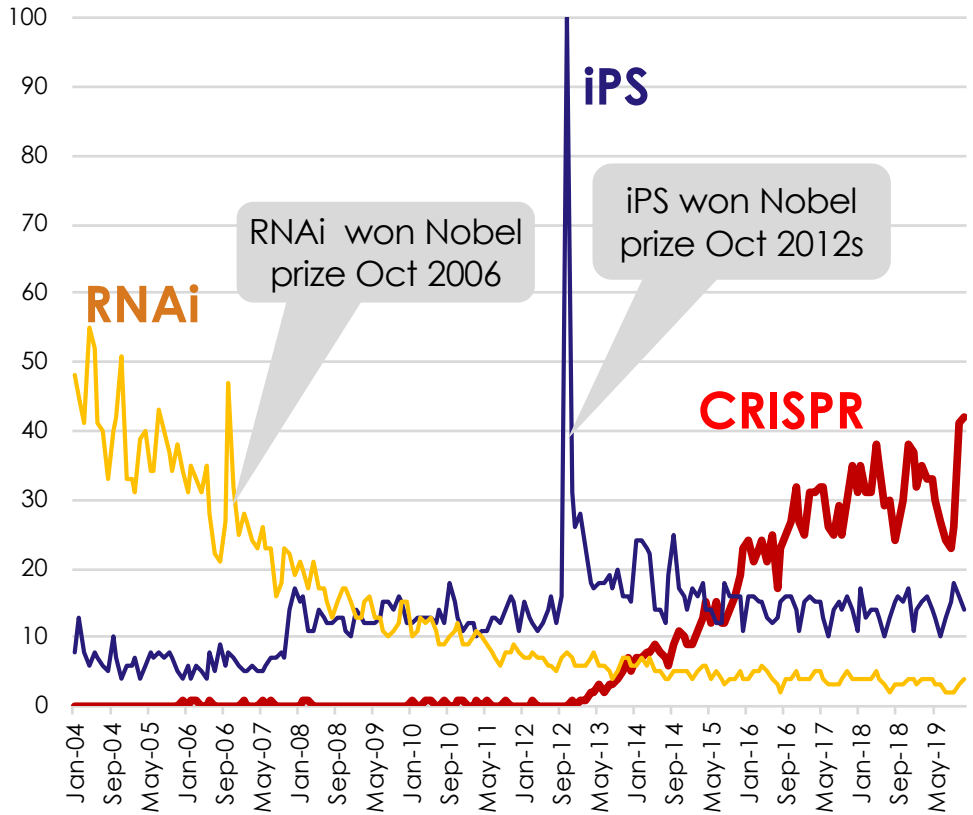
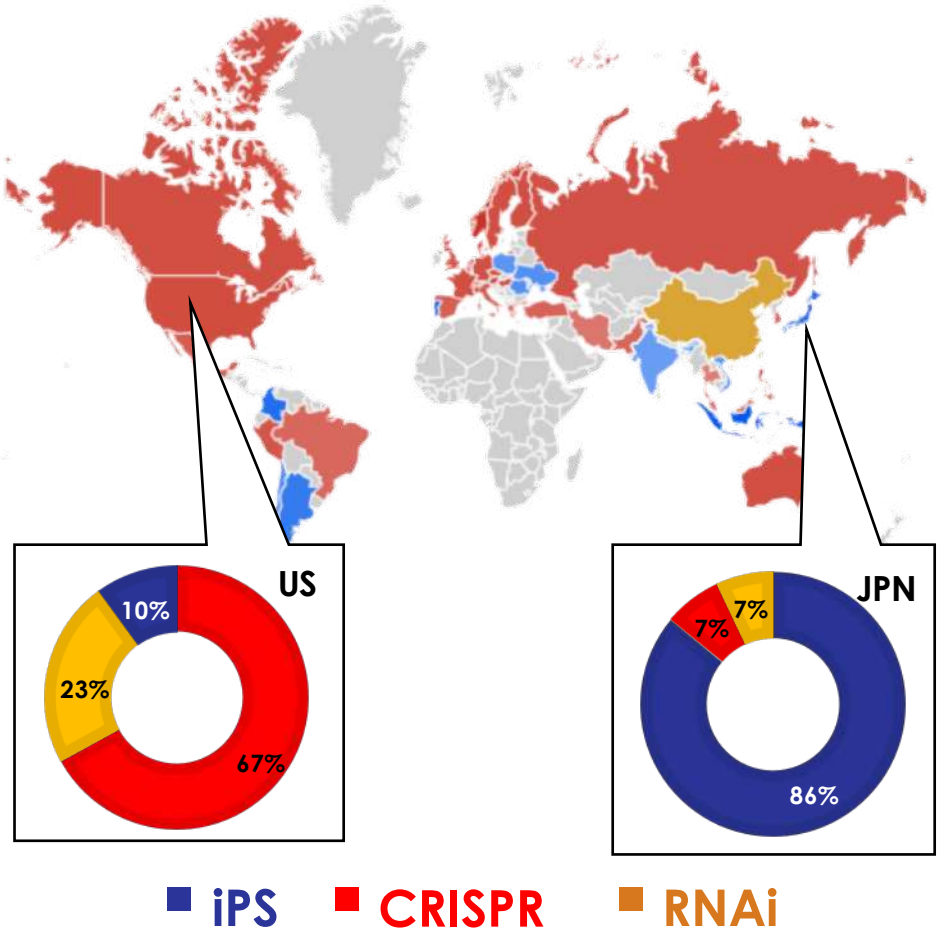
CRISPR gets more attention

Trends in Google keyword searches (world wide)



Geographic distribution of key word Google search (2005-2015)



Source : Google Trends
Note: Normalized by max of iPS at Oct 2012 as 100

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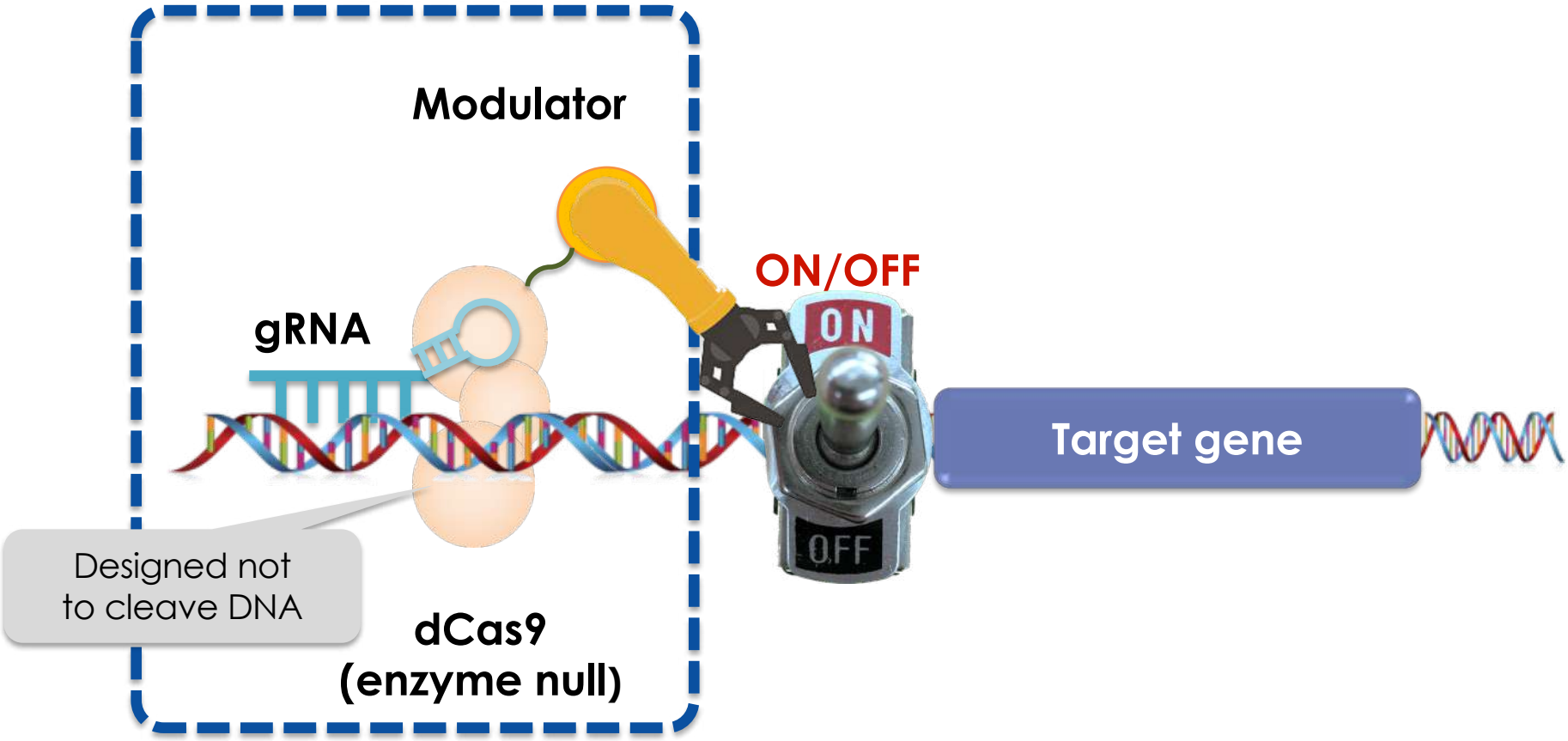
Non-cleaving CRISPR = CRISPR-GNDM®

Enables treatment of genetic disorders by controlling ON/OFF switch

CRISPR-GNDM (Guide Nucleotide-Directed Modulation) platform



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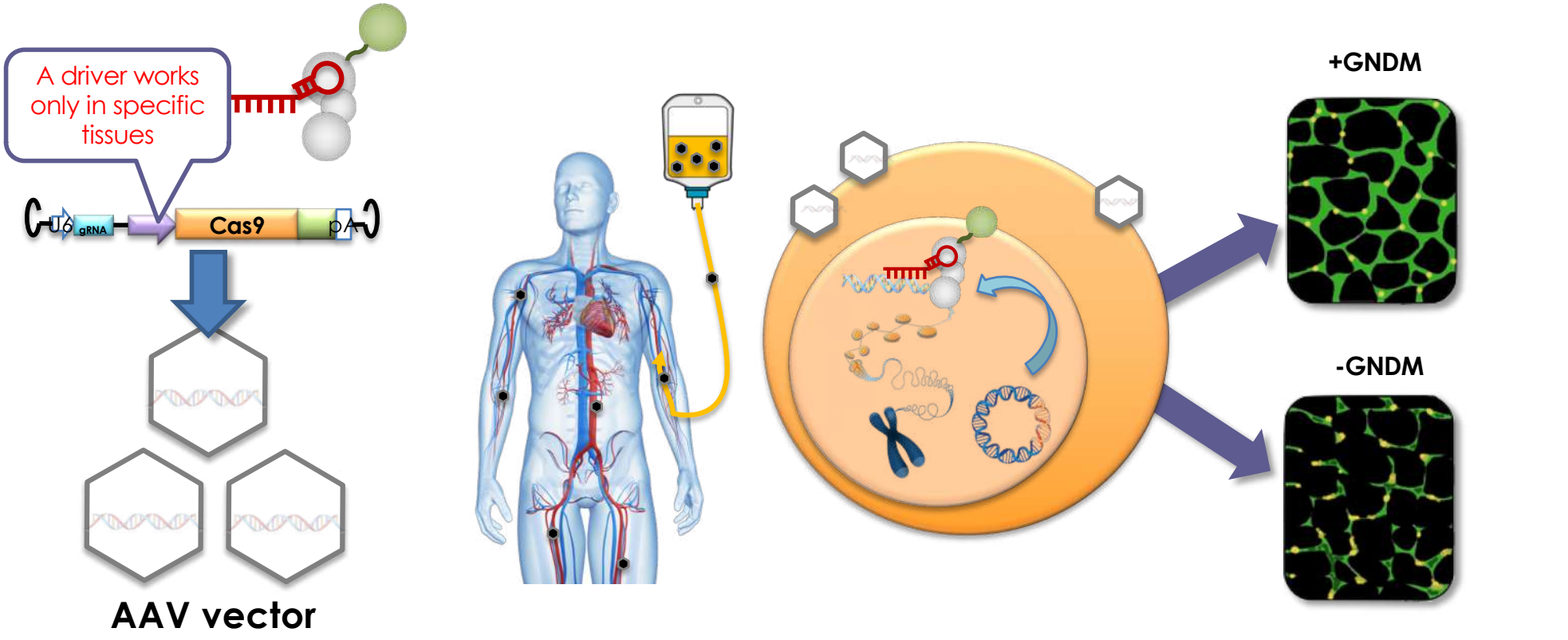
Delivery of CRISPR-GNDM® to target

Use AAV vector to deliver GNDM to target cell



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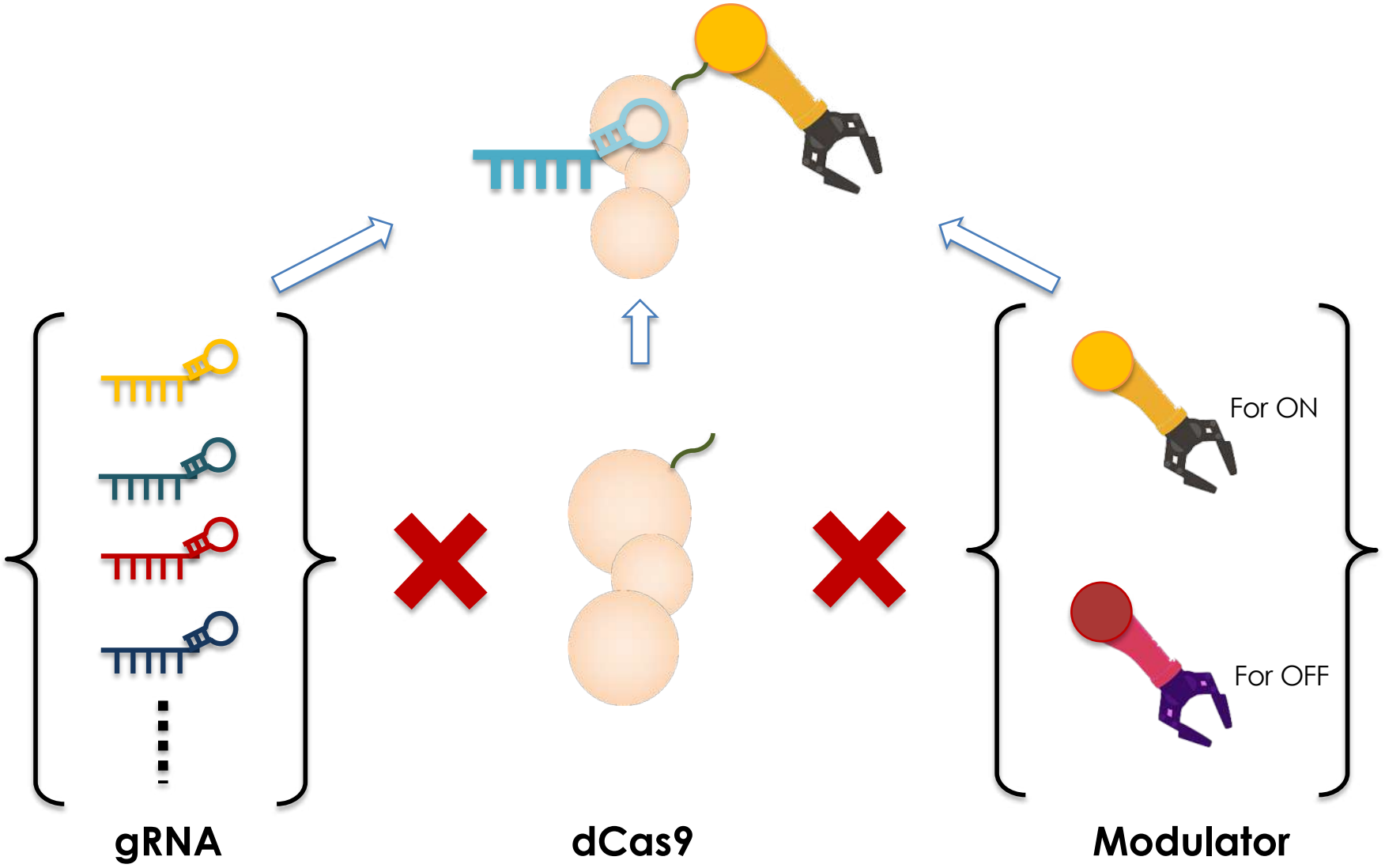
Scalability of GNDM

gRNA is the only variable which is unique for each target. Other components are off-the-shelf to assemble.



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MDC1A

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

- Frequency: **1 in 30,000***
- Inheritance Pattern: **Autosomal Recessive**
- Early onset: apparent at birth or within the **first few months of life**
- Symptoms:
  Severe muscle weakness;
  Lack of muscle tone (hypotonia);
  Little spontaneous movement;
  Joint deformities (contractures);
  Heart problems and seizures.
- Life expectancy:
  Because of the serious health problems that occur in this form of the disorder, many affected individuals **do not survive past adolescence**.
- Genetic cause: **LAMA2** mutation

MDC1A: merosin- deficient congenital muscular dystrophy type 1A
*Orphanet

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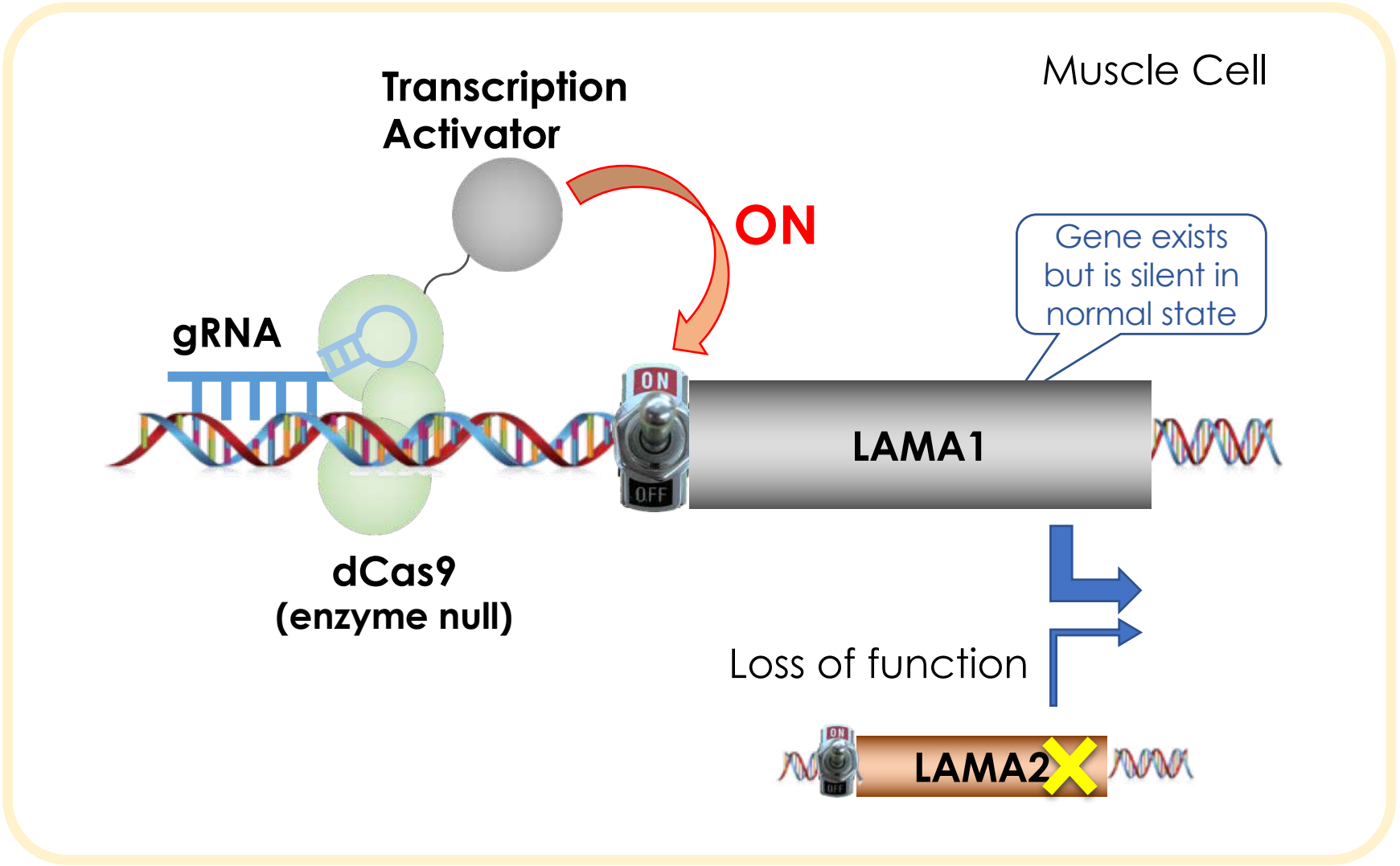
How GNDM cure MDC1A

GNDM upregulation of LAMA1 gene in skeletal muscle

CRISPR-GNDM targeting LAMA1



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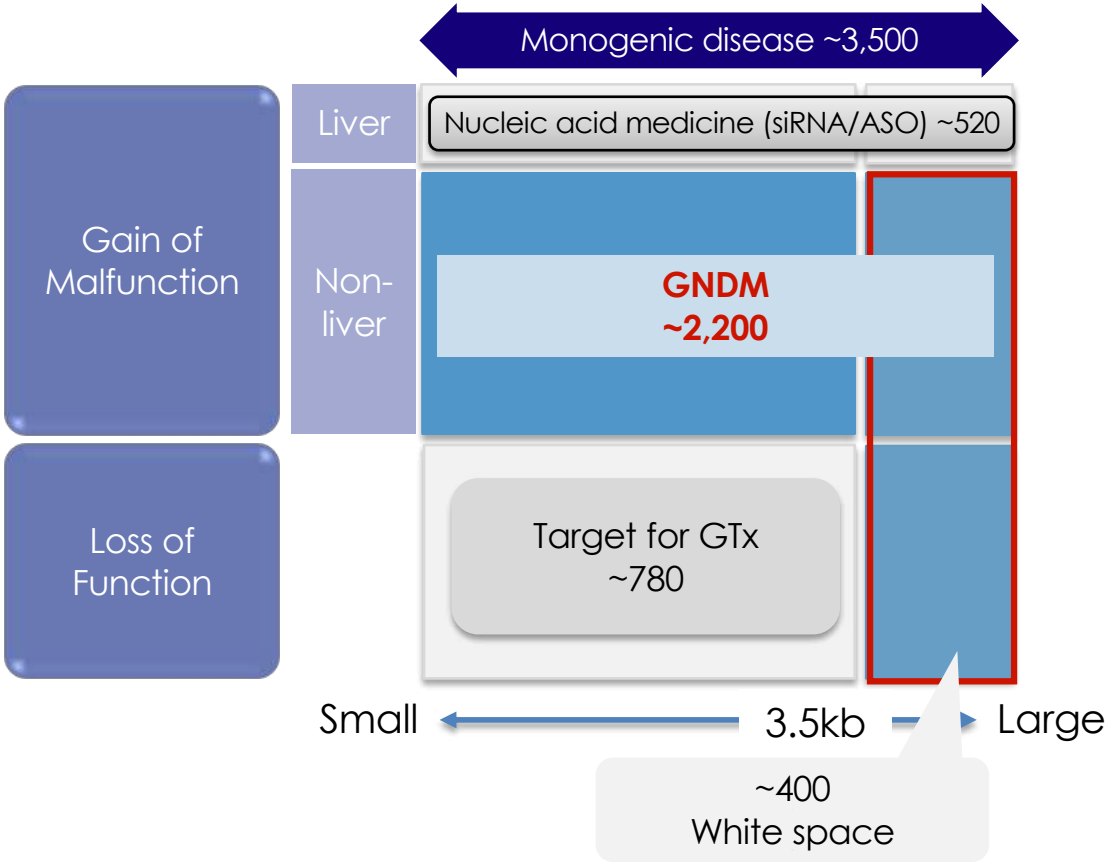
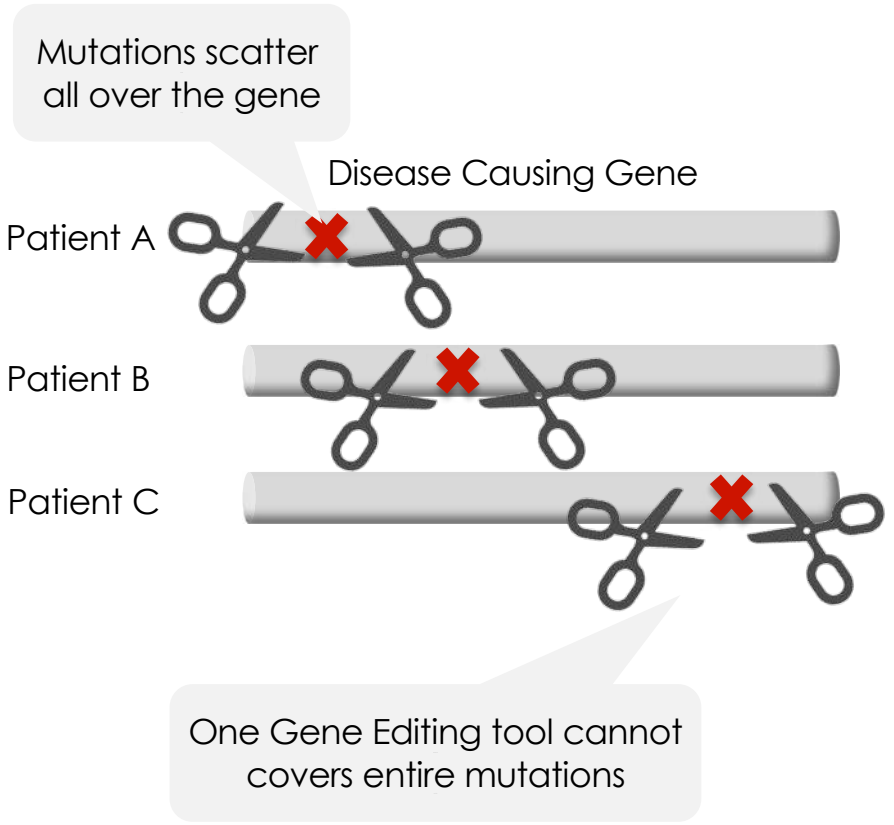
Target diseases of GNDM

GNDM enable us to reach differentiated genetic targets

Targets of GNDM



Limitation of Gene Editing



GNDM enable us to approach differentiated targets which cannot be reached by other gene therapies or gene editing

Source: Discovery Medicine、Science 2019

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Business Model

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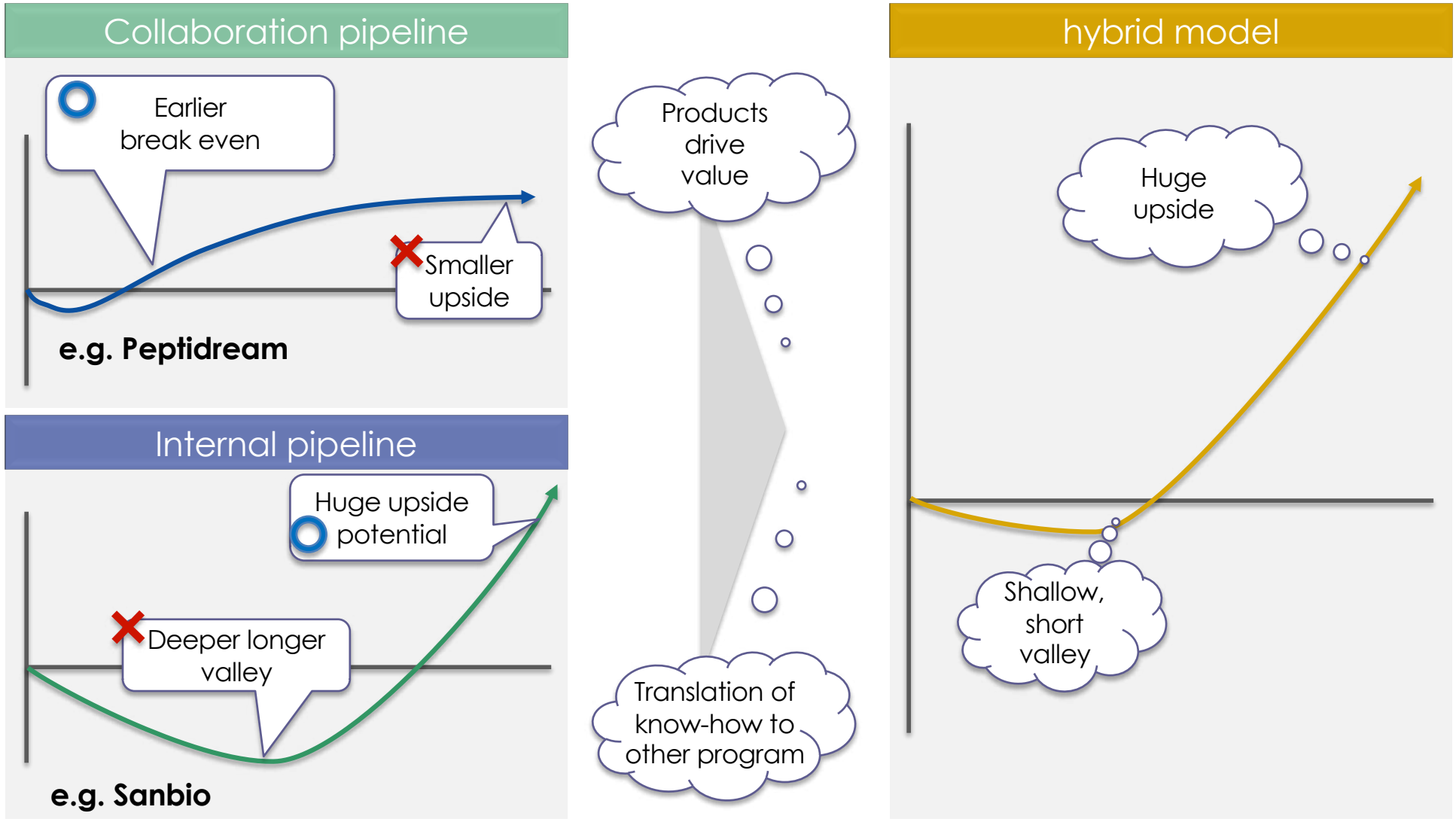
Modalis is pursuing a hybrid model

Combination of upside from internal pipelines and earlier cash stream from collaboration pipelines



* The above is only an image and does not suggest or guarantee our future performance.

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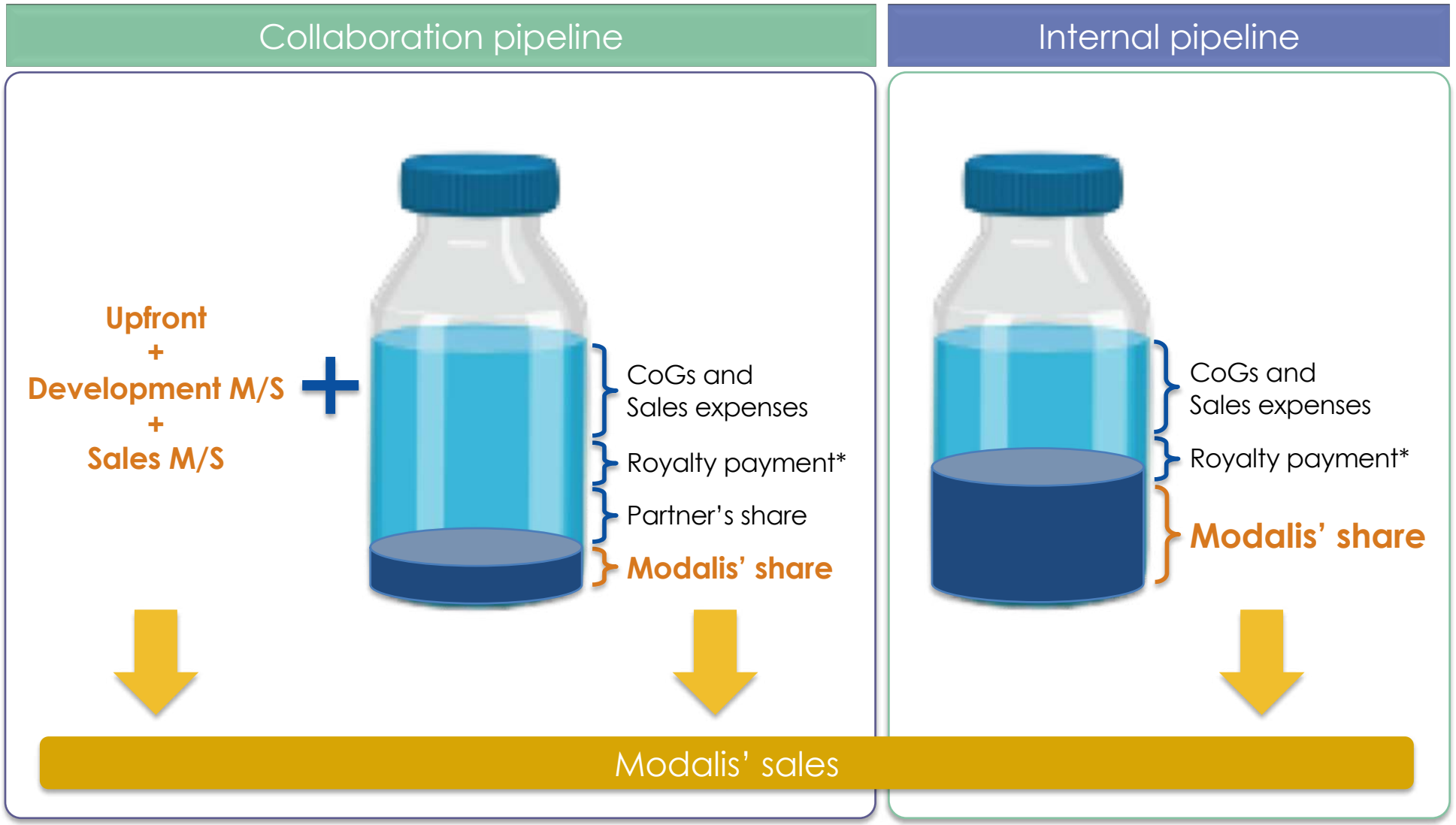
Risk - profit share model

While collaboration model brings earlier cash, internal pipeline has higher profit with higher risks



* : As a consideration for the licensed in intellectual property, a certain percentage of sales after market launch is paid to the intellectual property holding organization.

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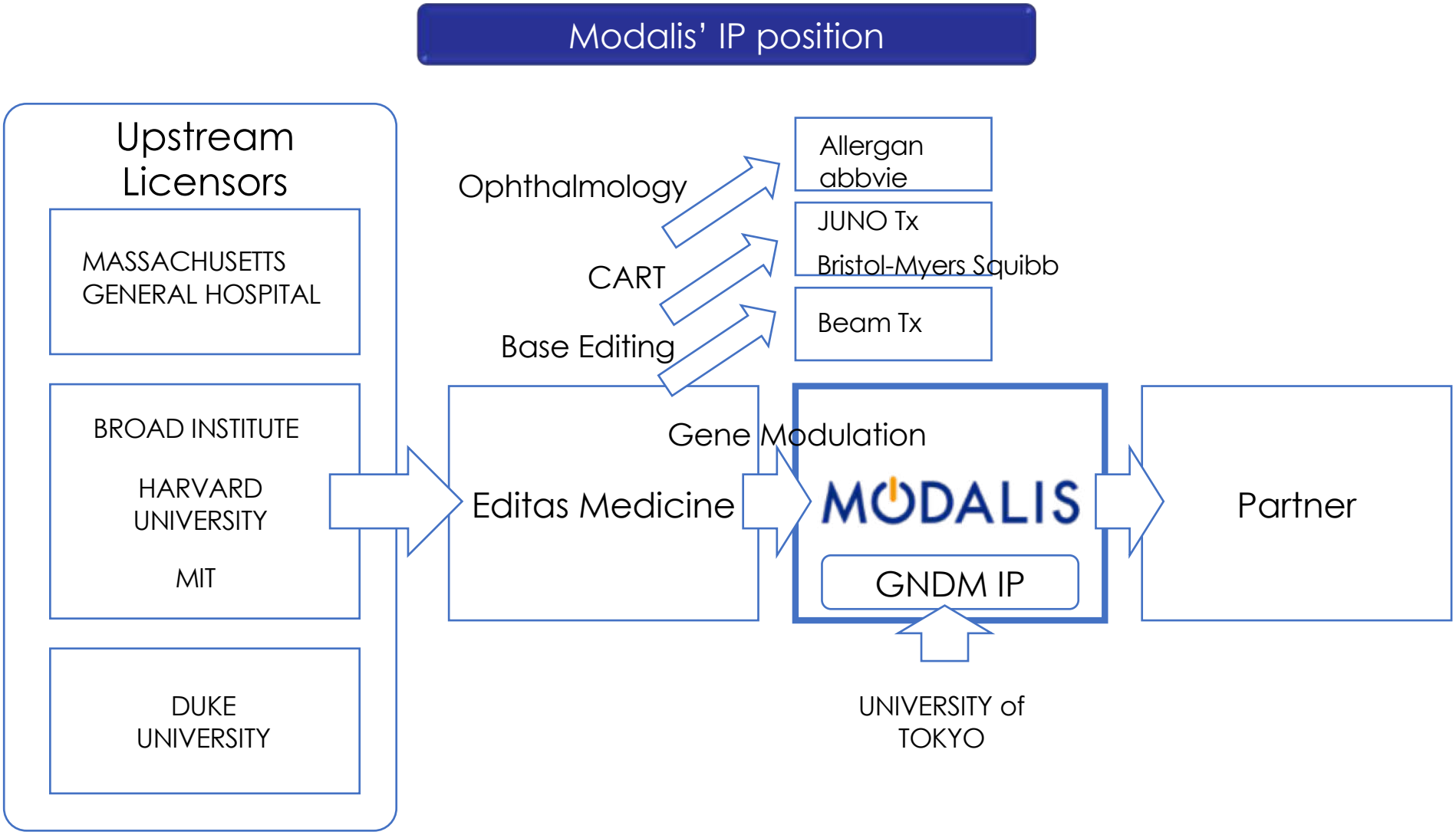
IP position

Established IP with foundational CIRPSR IP from Broad and engineered Cas9 from Univ of Tokyo



Source : disclosed information by each company

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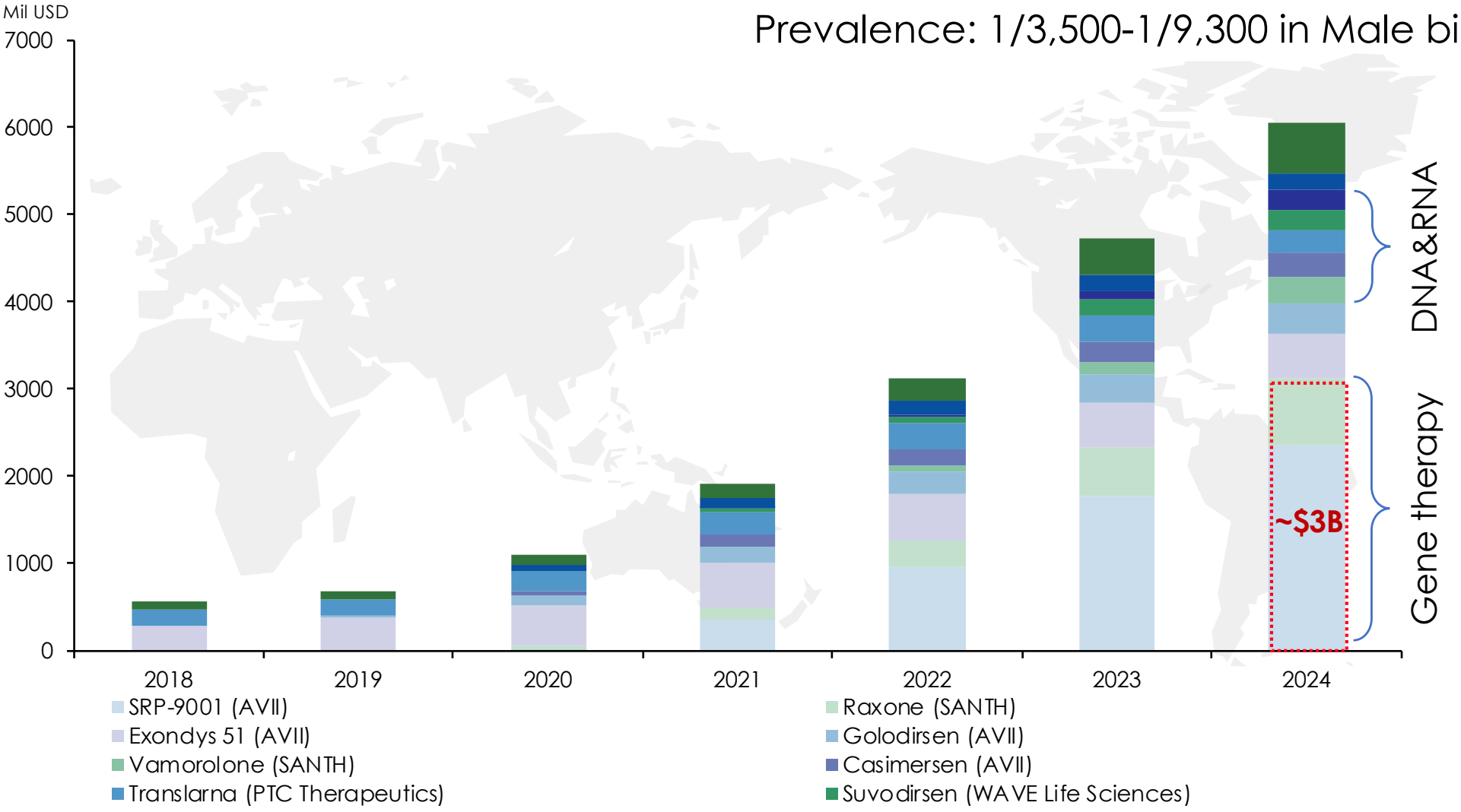
DMD market

Expected to be reaching $3B in 2024 driven by GTx

Prevalence: 1/3,500-1/9,300 in Male birth



Source : Evaluate Ltd and Orphanet. 2019 to 2024 are predicted sales

DMD: Duchenne muscular dystrophy

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Deal of CRISPR companies

3 editing companies have formed mega deal before entered into clinical trials

	Partner	Collaboration	Detail	Total deal size*
Editas Medicine Market Cap# : $1.7B (EDIT)	**JUNO Tx** **Bristol-Myers Squibb**	**3 target in CART**	Upfront $25M + R&D funding $22M + M/S $700M	**$ 747M**
	Allergan **abbvie**	**5 targets in Ophthalmology Incl. LCA10**	U/F $90M + MS・royalty	
Intellia Tx Market Cap# : $1.2B (NTLA)	**NOVARTIS**	**CART and HSC**	$10M（U/F） + $20M（tech transfer) + $20M（R&D funding) + $230.3M（M/S / 1 product) + ~15%（royalty) $13M Equity investment	**$ 293.3M**
			& 14 DDS patent license from Novartis	
	REGENERON	Option for **10 liver target**	$75M（U/F) + $135M（M/S / 1 product) + ~10%（royalty) $50M Equity investment@ IPO	**$ 260M**
CRISPR Tx Market Cap# : $4.5B (CRSP)	**VERTEX**	**Option to 6 targets**	$75M（U/F) + $30M（IP milestone) + $420M（MS / 1 target）+ royalty	**$ 525M**
	BAYER	**JV**	50:50 stake & $70M Equity investment	**$ 405M**
	CASEBIA	**Hematology** **Blindness** **Heart**	BAYER to CASEBIA $45M（U/F） + $255M（R&D funding)	
			CASEBIAからCRISPR $15M（U/F）、$20M（when get IP)	

Source: S1 data and press release of each company. # As of end Jun 24th * Aggregated amount of U/F, milestones and equity investment

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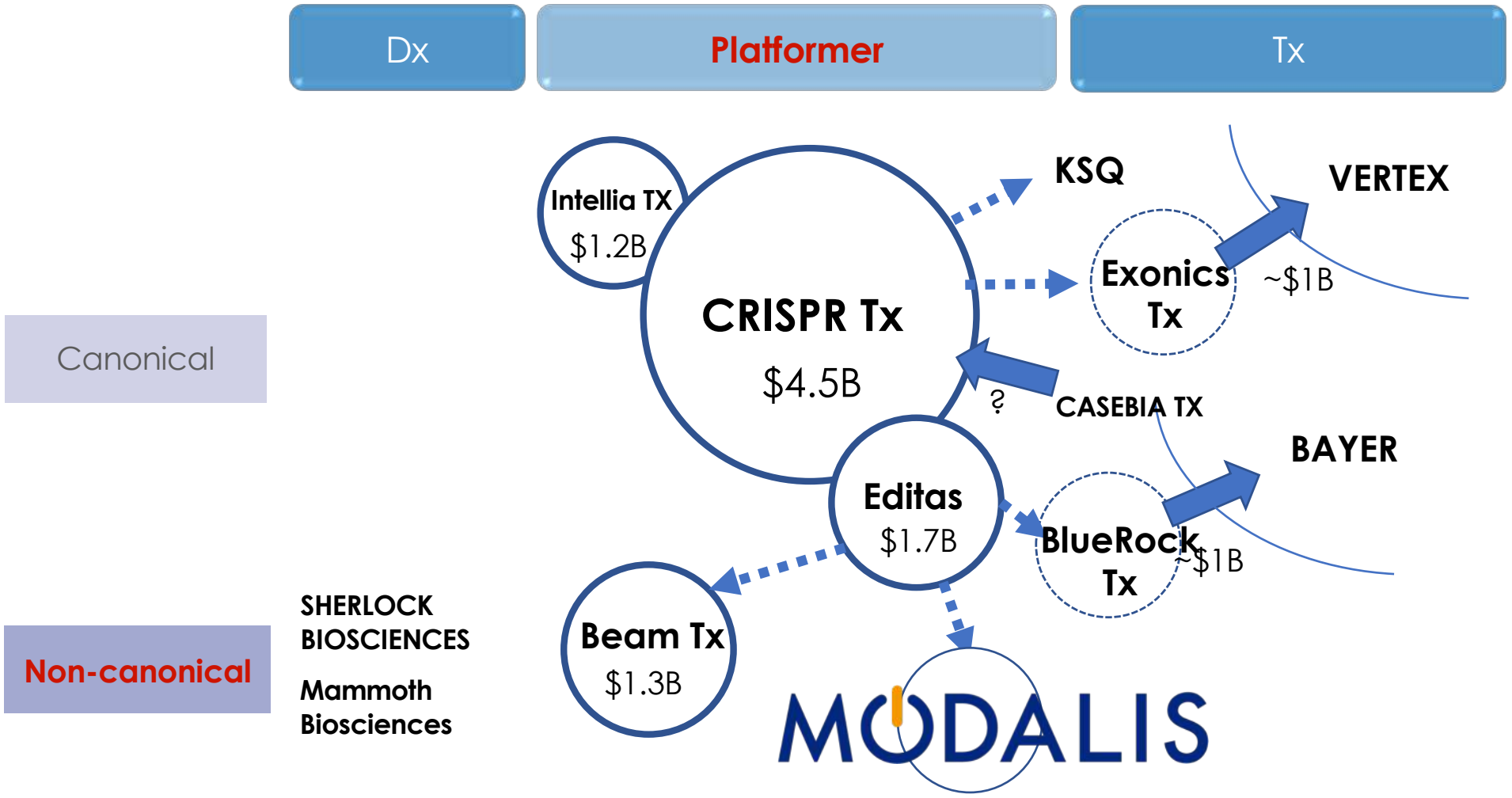
Major players in CRISPR field

Modalis established unique position in CRISPR companies, most of which reached >$1B



Source: stock info. The figures represent market cap as of Jun 24th or value at the time of acquisition. Dotted circle represent acquired companies

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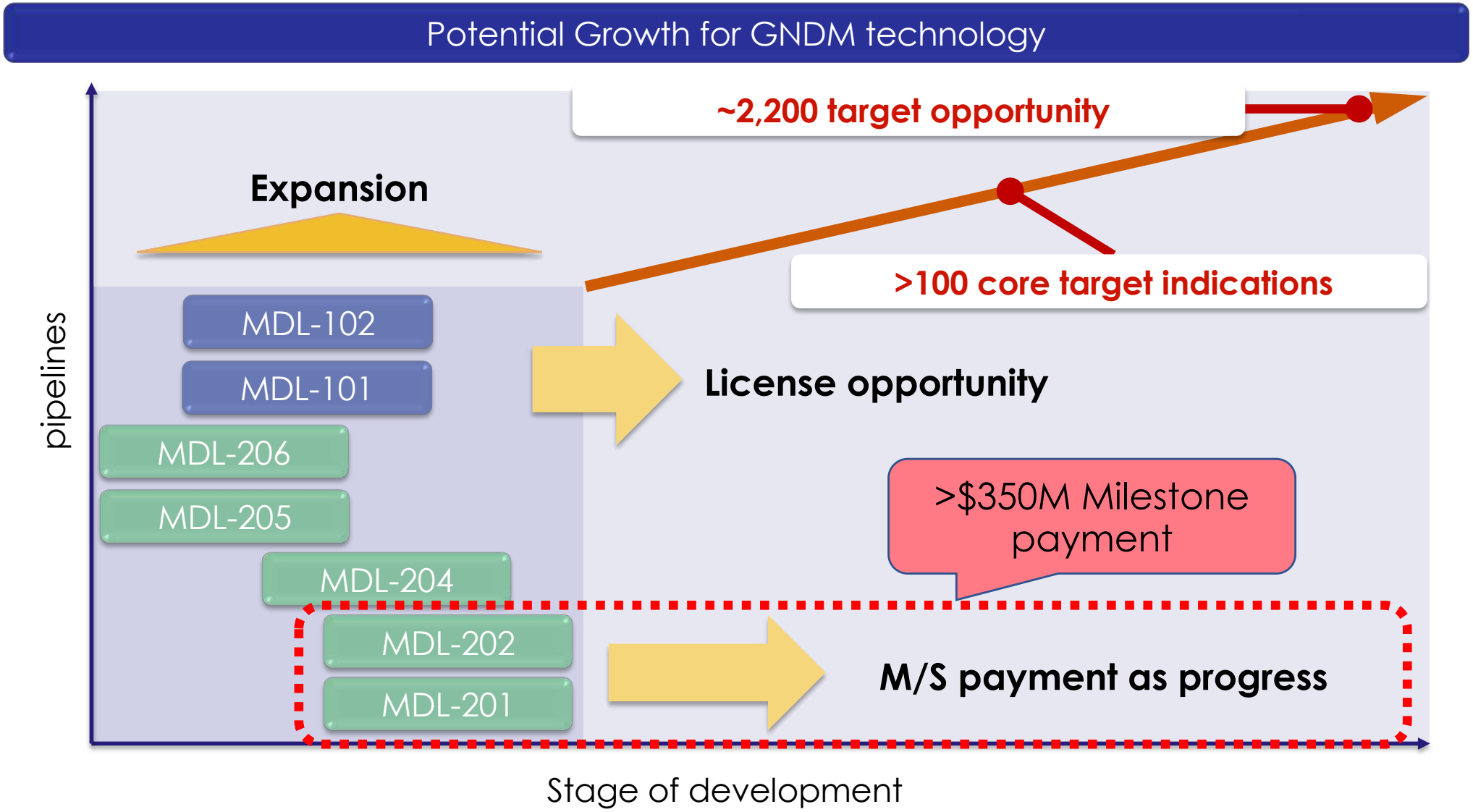
Growth Strategy

Growth Strategy
opportunity expands two dimensionally



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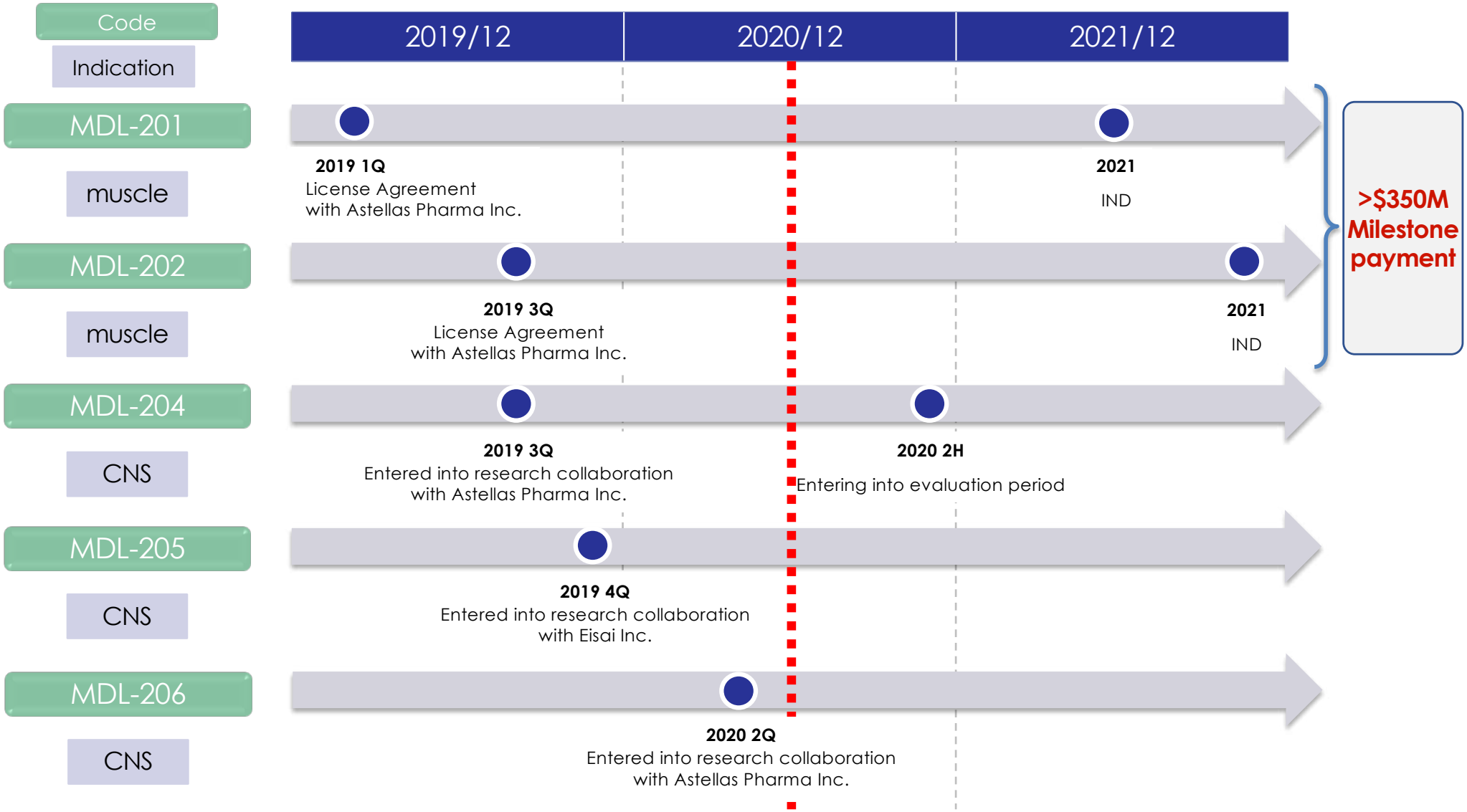
Collaboration pipeline

Achieved and expected Milestone events*



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

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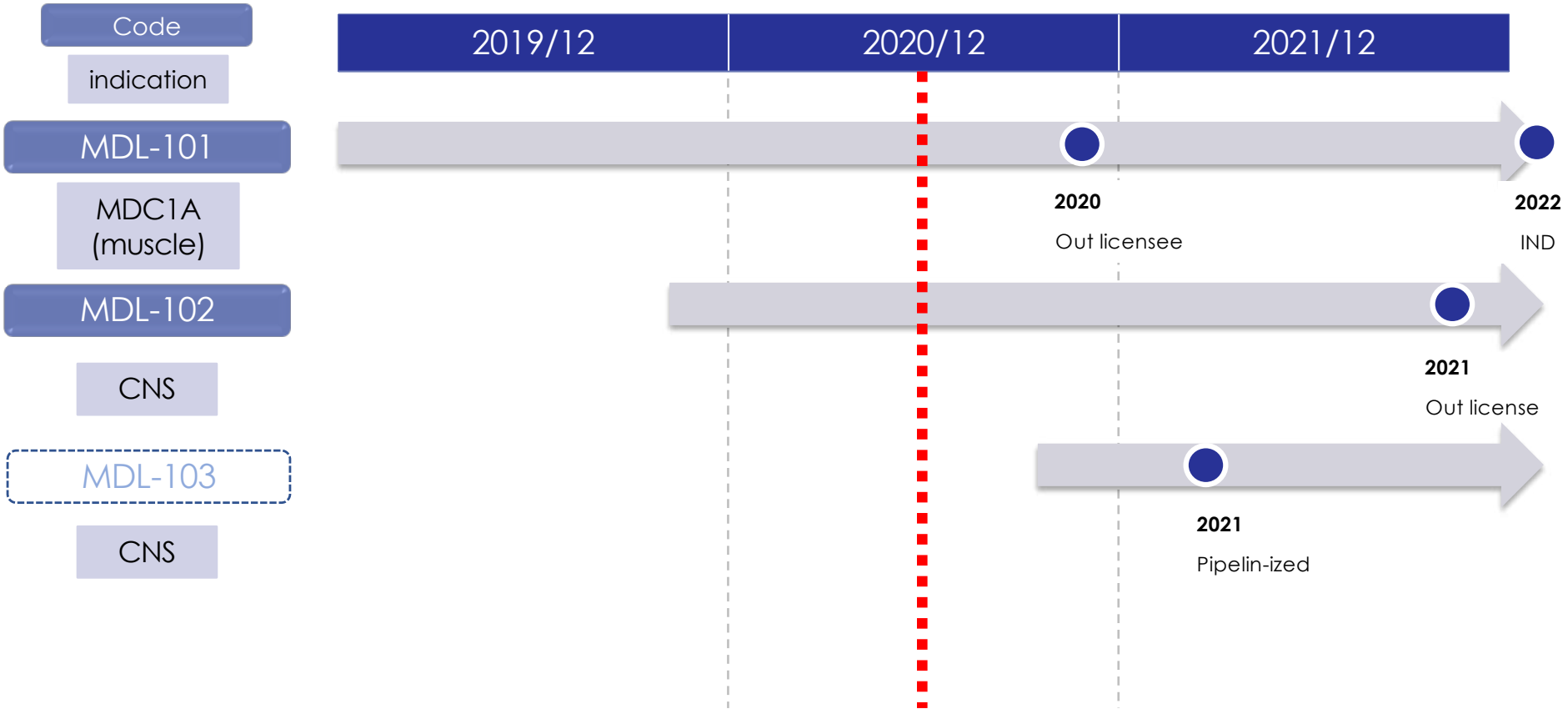
Internal pipeline

Expected milestones by end 2021*



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

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Advantages of GNDM in our business

- Unlimited upside potential
  - >100s potential indication that GNDM is superior to the other technologies
- Bye-bye, one-in-millions
  - Predictable result and timeline based on experience and streamlined research process
- Scalable
  - Experience in one indication is easily transplanted into the other

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Landscape of Listed Bio-ventures in Japan

First listing with CRISPR platform company

Business Model	Drugs / Therapies: Others	Small molecule	Protein / Peptide	Antibody	Regenerative / cellular medicine	Gene therapy / nucleic acid medicine	Gene Editing (CRISPR)
Drug discovery (Platformer)		**(4565) Sosei Group Mkt cap : 1,289B Yen**	**(4587) Pepti Dream Mkt cap : 6,345B Yen**				**MODALIS (4883) Modalis**
Drug discovery (Products)	Nano Carrier MED RX Oncolys BioPharma	CARNA BIOSCIENCES MEDICINOVA RaQualia CanBas DWTI Kubota Delta-Fly Pharma GNI SymBio Solasia	JCR OTS 3D MATRIX BrightPath Bio StemRIM Gene Techno Science	CHIOME Bioscience	SanBio Healios J-TEC CellSeed	Anges RIBOMIC	
analysis/contract processing services	SNBL HMT Phoenix Bio				Takara Bio MEDI NET REPROCELL CellSource		

Source: Prepared by Modalis Tx based on information disclosed by bio ventures listed from 1990 to date. The above chart classifies drugs and therapies based on main pipelines.
Market cap is as of Jun 24th

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Appendix

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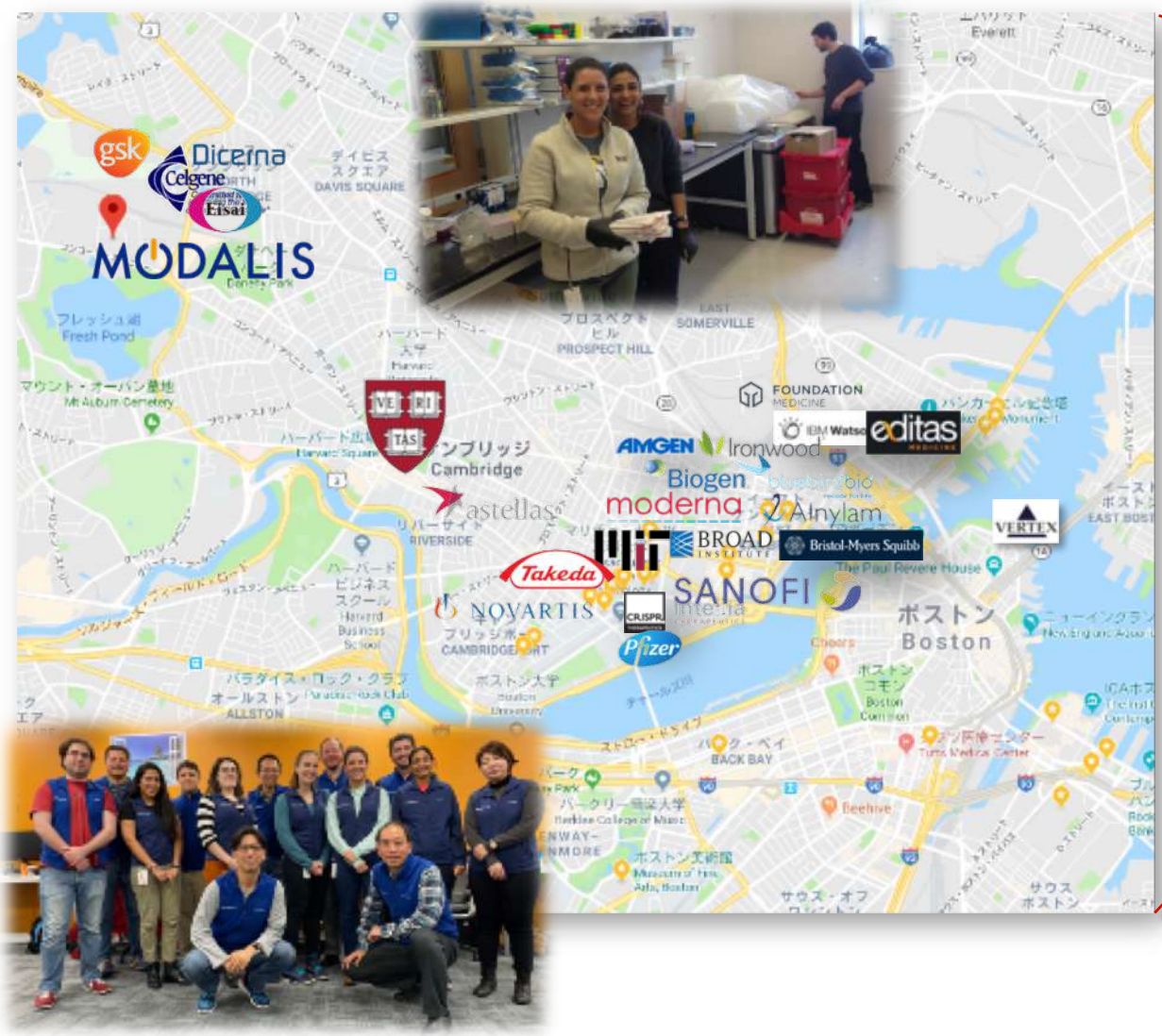
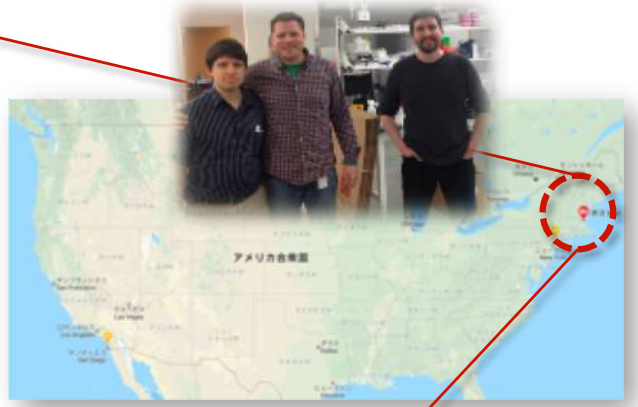
Operated in Cambridge MA

The center of life-science





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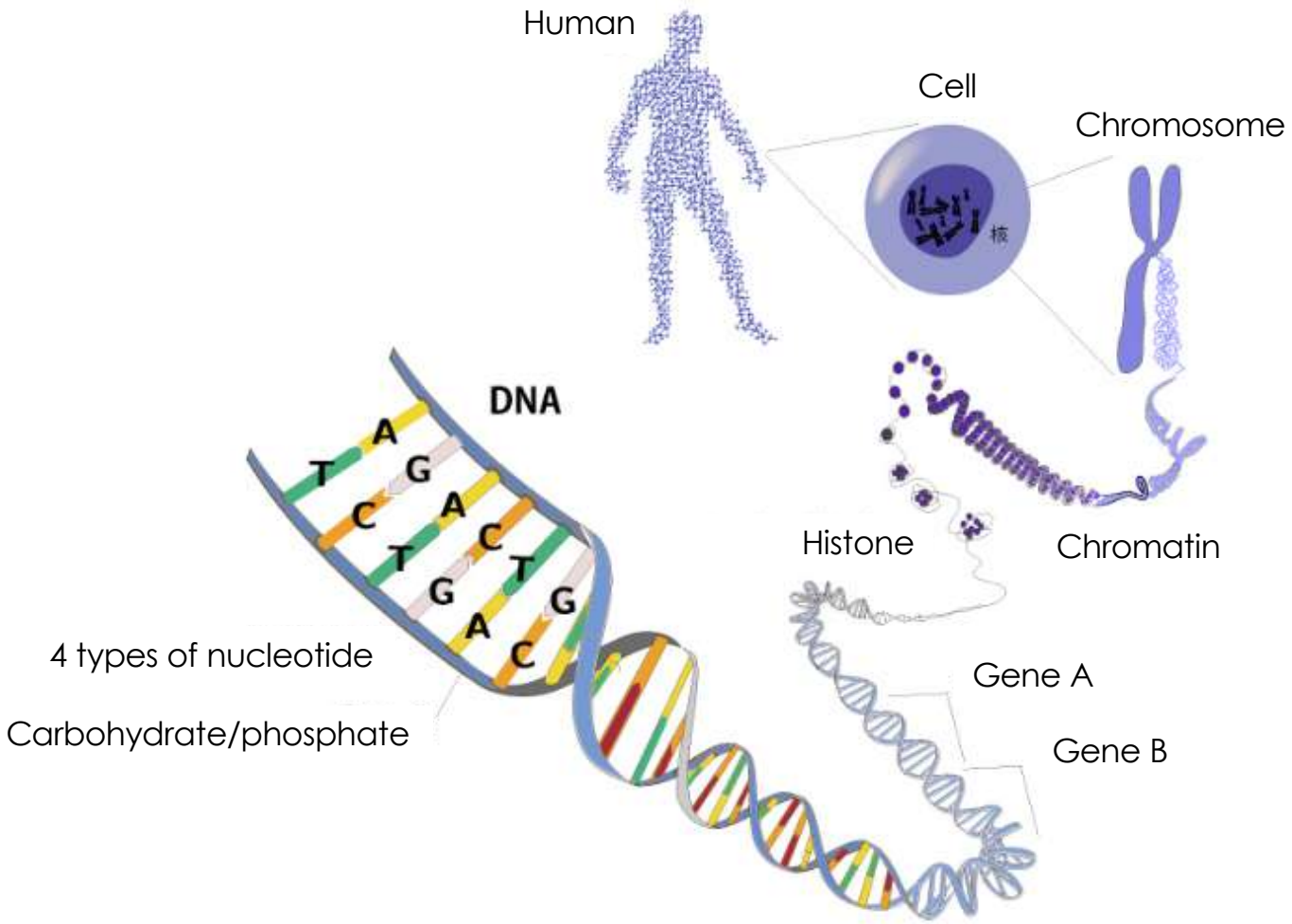
Genome, Gene, DNA

Gene is a meaningful unit (program) consisting of DNA (code) within the genome (system)



All cells in the body have the same DNA in all cell types

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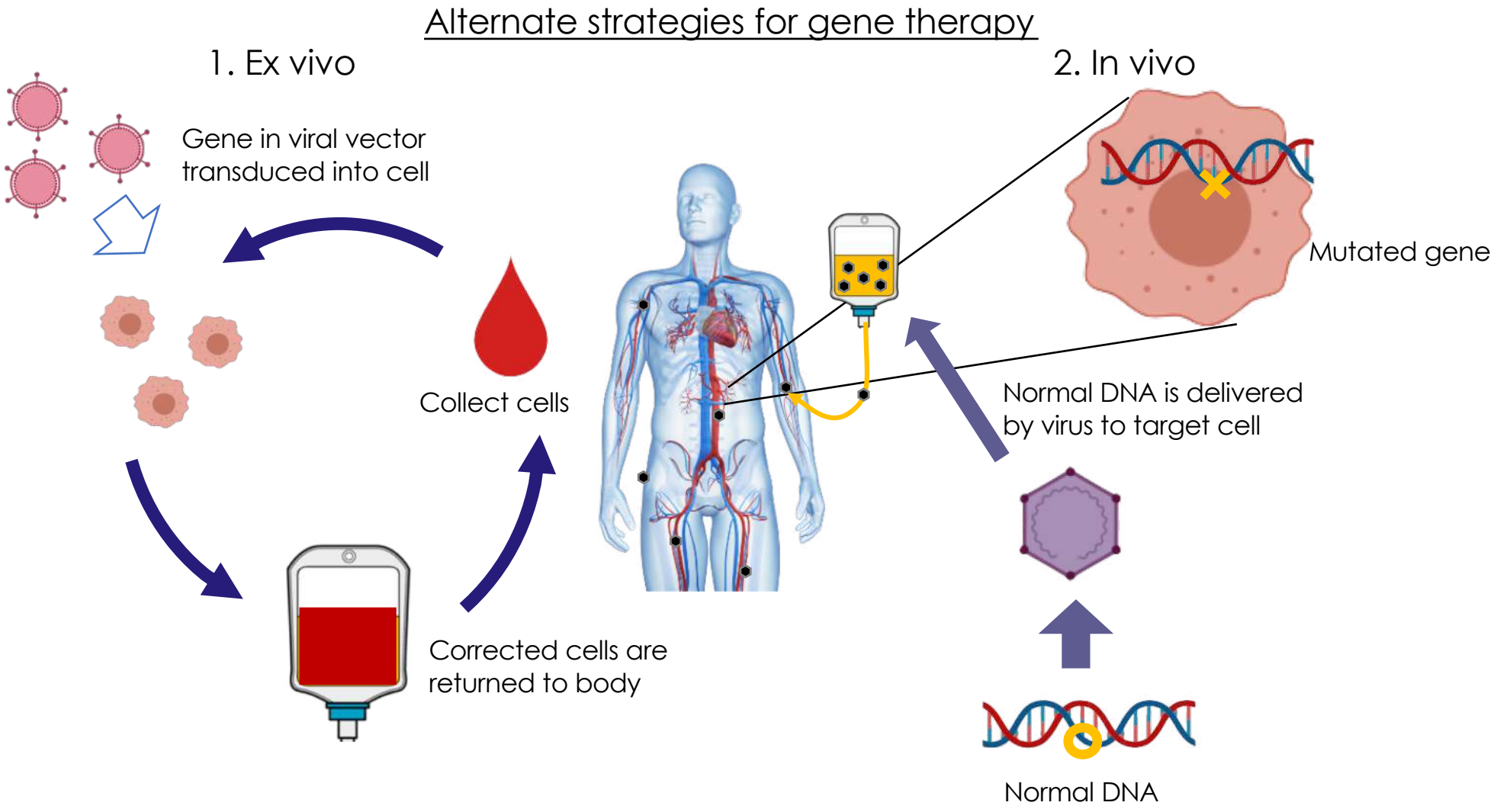
Delivery of gene therapy

Use of viral vector to deliver DNA to cell *ex vivo* or *in vivo*



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Comparison of GTx technologies

	Gene Therapy (broad sense)					
	GTx (narrow sense)	Gene Editing				
Basal technology	-	ZFN	TALEN	CRISPR	CRISPR	CRISPR
Molecule design	-	Complicated	Complicated	**Simple**	**Simple**	**Simple**
Mode	Gene Transduction	Gene Editing Gene modulation	Gene Editing	Gene Editing	Base Editing	Gene modulation
Delivery method	AAV/LNP	AAV	AAV	AAV/LNP	AAV/LNP	AAV
DNA cleaving	Yes	Yes	Yes	Yes	**No**	**No**
Max size of target gene	<~3.5kb	**No limit**	**No limit**	**No limit**	**No limit**	**No limit**
Companies	**AUDENTES Tx** **Spark Tx** **AveXis** **SAREPTA Tx** **REGENXBIO**	**Sangamo Tx**	-	**Editas Medicine** **Intellia Tx** **CRISPR Tx**	**Beam Tx**	MODALIS

Source: Modalis by public information

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Glossary（1/2)

Page	Word	Explanation
P9	CNS	Central Nervous System comprises the brain and spinal cord.
P9	MDC1A	A group of neuromuscular disorders that begin at birth or infancy and are characterized mainly by hypotonia, muscle weakness and muscle wasting. Caused by mutations in the LAMA2 gene. Inherited in an autosomal recessive manner.
P21	GTx	Gene Therapy
P24	ZFN（Zinc Finger)	Small protein structural motif that is characterized by the coordination of one or more zinc ions (Zn2+) which stabilize the structure. Engineered ZF arrays fused to a DNA cleavage domain (usually the cleavage domain of FokI) are used as a gene editing tool.
P24	TALEN	Transcription Activator-Like Effector Nucleases: restriction enzymes that can be engineered to cut specific sequences of DNA. They are made by fusing a TAL effector DNA-binding domain to a DNA cleavage domain.
P24	gRNA（guide RNA)	18-22nt RNA which is complementary (forming A-T or G–C pairs) and bind to target DNA sequence
P26	dCas9	Form of Cas9 enzyme which lacks activity to cut DNA
P27	AAV vector	Adeno-associated virus (AAV) is a small virus that infects humans and some primate species. AAV is not currently known to cause disease and is used for delivery of gene therapy tools to cells for therapeutics.

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Glossary (2/2)

Page	Word	Explanation
P31	Gain of Malfunction	A type of mutation in which the altered gene product possesses a harmful molecular function or a harmful pattern of gene expression. Gain-of-function mutations are almost always Dominant or Semi-dominant
P31	Loss of function	Also known as inactivating mutations, which result in the gene product having less or no function
P31	siRNA	Small interfering RNA (siRNA) is a class of double-stranded RNA molecules, 20-25 base pairs in length, and operating within the RNA interference (RNAi) pathway. They interfere with the expression of specific genes with complementary nucleotide sequences by degrading mRNA after transcription thus preventing translation.
P31	ASO	A synthetic strand of nucleic acid (DNA, RNA or a chemical analogue) that binds to the messenger RNA (mRNA) produced by that gene and inactivates it, effectively turning that gene "off".
P36	Duchenne muscular dystrophy(DMD)	Genetic disorder characterized by progressive muscle degeneration and weakness. One of nine types of muscular dystrophy. DMD is caused by an absence of *dystrophin*, a protein that helps keep muscle cells intact.
P47	Histone	Proteins found in eukaryotic cell nuclei that package and order the DNA into structural units.
P47	Chromatin	Complex of DNA and protein found in eukaryotic cells. Primary function is packaging very long DNA molecules into a more compact, denser form.
P48	Ex vivo / In vivo	Ex vivo: transduction of the therapeutic gene into patient-derived somatic cells, followed by subsequent transplantation back into the patient. In vivo: delivery of cargo (e.g. virus vector) and therapeutic components to body

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