



**Veritas In Silico**

Bringing new hope with mRNA-targeted drugs

FY2024 1H

# Interim Financial Results

**Veritas In Silico Inc.**

Ticker code: 130A

**August 8, 2024**



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In case of discrepancy, the Japanese original shall prevail.



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## Highlights for the first half of FY2024

### **Platform business for mRNA-targeted small molecule drug discovery performs well**

Joint drug discovery research is underway with four pharmaceutical companies utilizing the ibVIS® drug discovery platform. In the first half of the current fiscal year, the company achieved milestones and obtained several small molecule compounds with targeted profiles through cell-based assay.

### **Currently developing a structure to create in-house pipelines to transition to a hybrid-type business**

We resumed research on our proprietary nucleic acid drugs.

We are working toward creating nucleic acid drug pipelines for acute renal failure and amyotrophic lateral sclerosis (ALS).

### **Business revenue for the interim period was 115 million yen, Profit for the period was in the red.**

Business revenue totaled 115 million yen due to research support funds and milestone payment, etc.

Interim net loss was 90 million yen due to business expenses of 182million yen, listing-related expenses of 12million yen, etc.

### **Forecast for FY2024 remains unchanged**

No change from the February 2024 forecast for FY12/2024

Aiming to secure earnings by achieving milestones and acquiring new contracts during the second half of the fiscal year



# Our founder's background making mRNA-targeted small molecule drug discovery a reality

After the company's establishment, Nakamura fully leveraged his background to launch a full-fledged mRNA-targeted small molecule drug discovery business. He successfully raised three rounds of funding and achieved IPO in February 2024



Representative director and CEO  
**Shingo Nakamura, PhD**

Extensive operational experience ranging from drug research to business development, sales, management, and investment in biotech companies



## ~2003 Scientific background

Shingo studied **informatics** at Nagoya University and received his PhD in **organic chemistry**. He later studied **RNA biology** during his postdoctoral fellowship at Yale University. The concept of mRNA-targeted small molecule drug discovery was shaped by his diverse scientific background.

## 2003-2011 Pharmaceutical industry research

Shingo conceived the idea of mRNA-targeted small molecule drug discovery at Takeda in 2003 and led an in-house project for seven years.

He filed the **world's first business model patent for mRNA-targeted small molecule drug discovery** in 2004.

## 2011-2015 Business background

Shingo gained hands-on business experience working as a sales and marketing manager for Dow Chemical and later as a business development director for Catalent\*.

## 2015-2017 Experience as an investor

Shingo worked as a venture capitalist at the Innovation Network Corporation of Japan (INCJ) and served as a director of the board of biotech companies which he invested in, gaining hands-on experience in company establishment and management.

\*A global contract development and manufacturing organization (CDMO) headquartered in New Jersey, USA, that provides delivery technologies, drug manufacturing, biologics, gene therapies, and consumer health products for pharmaceutical and biotech companies

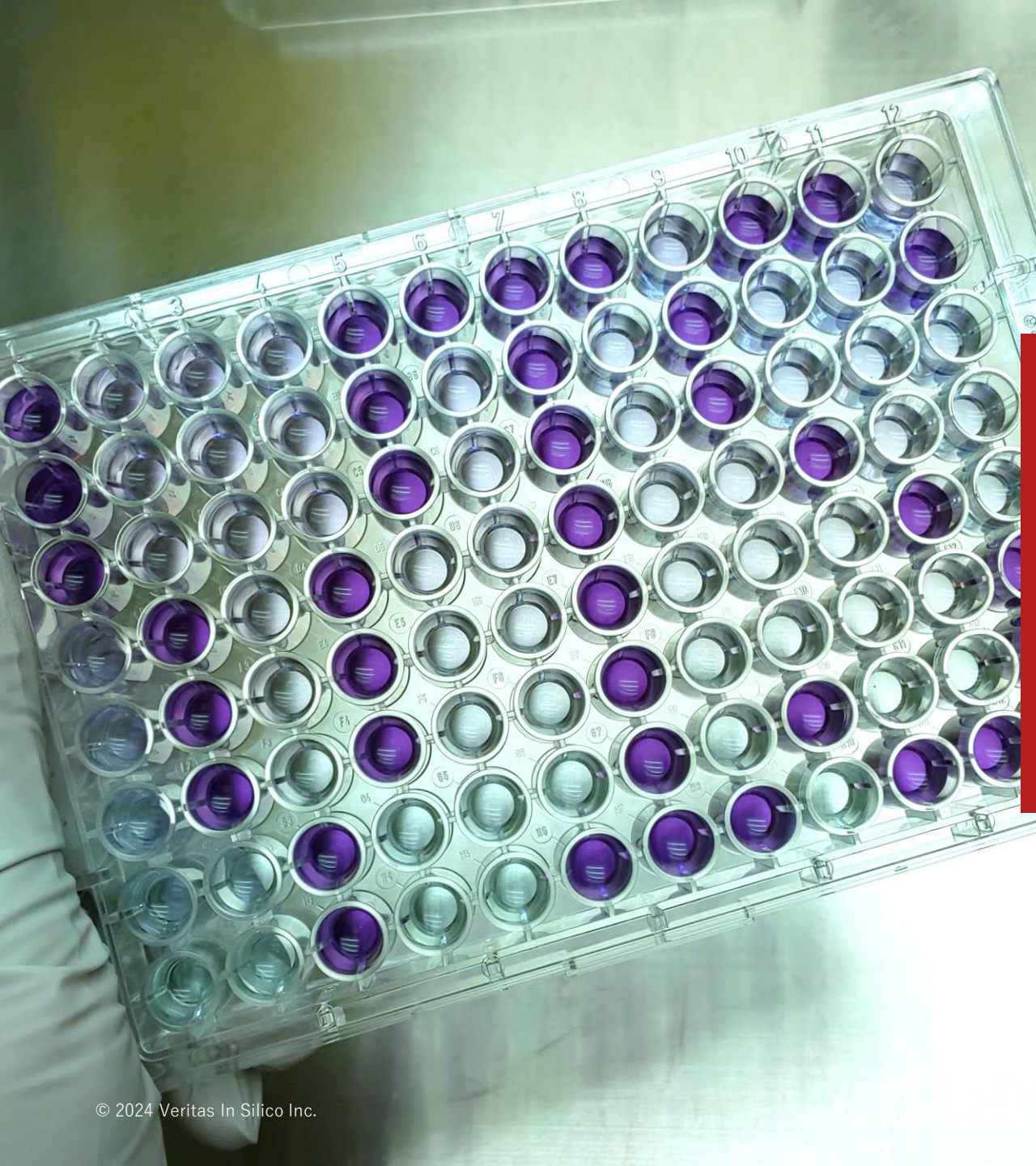




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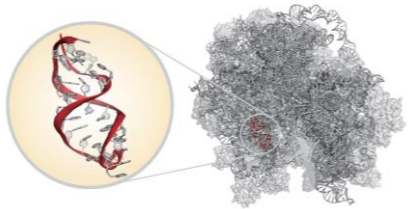


# 1 Business Highlights



## Business highlights for the first half of FY2024

### ▼February

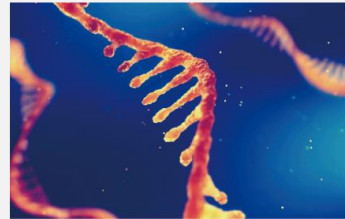


#### Review Article on the Use of Artificial Intelligence (AI)

Our Senior Scientist published a review paper discussing how AI can maximize the potential of RNA-targeted small molecule drug discovery.

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### ▼March



#### Efforts to Create In-house Pipeline of Nucleic Acid Drugs

Started in-house research to obtain more efficient and active nucleic acid drugs prior to the joint research with Mitsubishi Gas Chemical

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### ▼June



#### Milestone Achieved

Achieved first research milestone in drug discovery collaboration with Takeda to create mRNA-targeted small molecule drugs for multiple diseases

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### ▼June



#### Steady Progress in Compound Discovery

Obtained several small molecule compounds with target profiles through cell-based assays in a joint drug discovery project with RaQualia aimed for the discovery of anti-cancer drugs

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## Reached milestone in joint drug discovery research and licensing agreement with Takeda

2024.06.19

Press Release

### Veritas In Silico Announces Milestone Achievement in Collaborative Research with Takeda

**Tokyo, Japan, June 19, 2024** –Veritas In Silico Inc. (“VIS”) announced today that it has reached a milestone in the collaborative research with Takeda Pharmaceutical Company Limited (“Takeda”), which is being conducted under the drug discovery collaboration and license agreement signed on June 23, 2023.

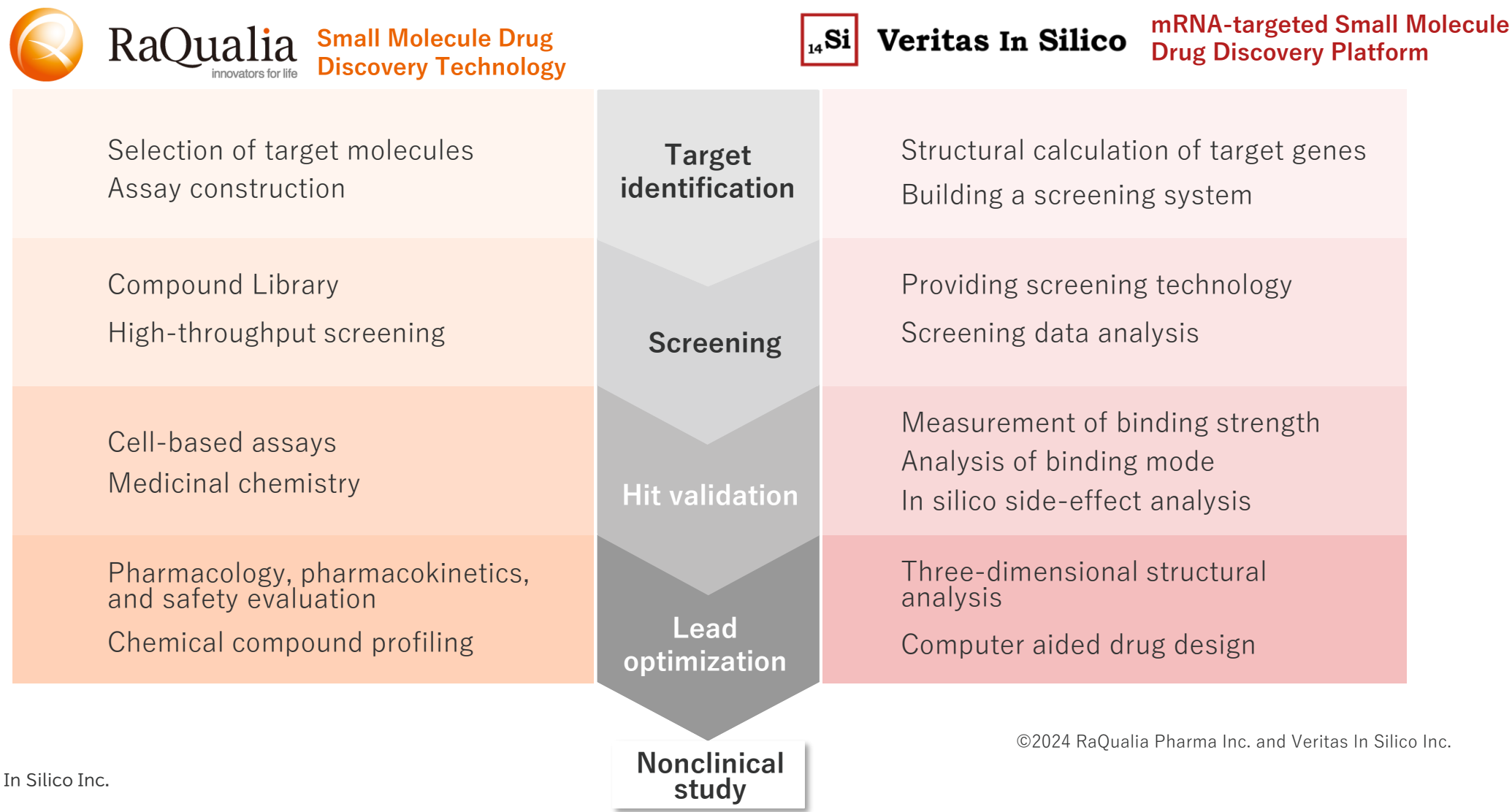
The collaborative research utilizes VIS’s proprietary drug discovery platform, ibVIS<sup>®</sup>, to create small molecule drugs that target mRNA for multiple genes related to diseases selected by Takeda. Since the signing of this agreement, both companies have been jointly searching for novel small molecule compounds that act on the mRNA of the target genes and have now achieved the criteria for the milestone set forth in the agreement. Veritas In Silico will receive a milestone payment and the revenue is supposed to be recognized in the second quarter of the fiscal year ending December 31, 2024.

The impact of achieving this milestone on the financial performance of Veritas In Silico has already been factored into a forecast for the fiscal year ending December 31, 2024, announced on February 13, 2024. It is expected to account for approximately less than 10% of the total revenues (676 million yen forecast).




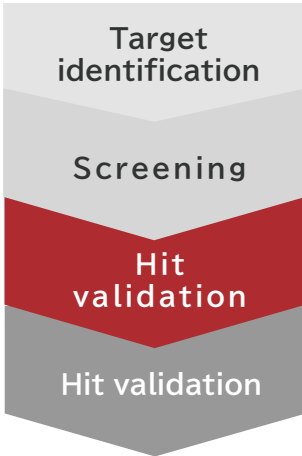



# Steady progress in compound acquisition in joint drug discovery research with RaQualia

We acquired several small molecule compounds with target profiles cell experiments in a joint drug discovery project with RaQualia aimed at creating anticancer drugs, by leveraging the strengths of each company's technological capabilities





# Proven track record in joint drug discovery projects with pharmaceutical companies

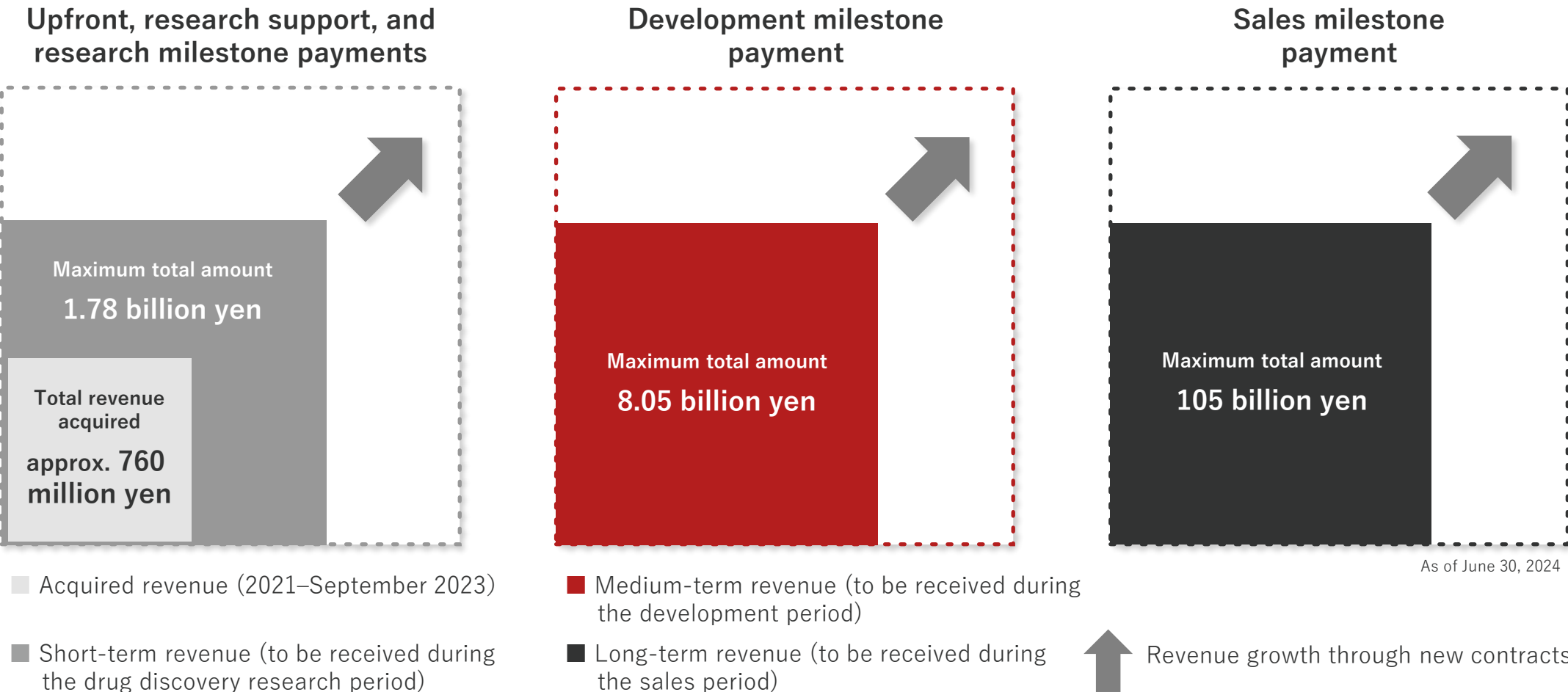
Major partners (Arranged according to timing of agreement)	Timing of agreement	Target disease	Stage of drug discovery research	Financial conditions
 Innovation by Chemistry	July 2021	Undisclosed		<ul style="list-style-type: none"><li>• Contract amount undisclosed</li><li>• VIS obtains rights to the compounds*</li></ul>
	November 2021	Infectious diseases, psychiatric and neurological disorders		<ul style="list-style-type: none"><li>• Up to 85 billion yen contract</li><li>• Royalties</li></ul>
 RaQualia <small>innovators for life</small>	December 2022	Cancer		<ul style="list-style-type: none"><li>• Contract amount undisclosed</li><li>• Royalties</li></ul>
	June 2023	Undisclosed		<ul style="list-style-type: none"><li>• Contract amount undisclosed</li><li>• Royalties</li></ul>
The most advanced projects in joint drug discovery research with pharmas are in the hit validation stage				

\*In the joint drug discovery research with Toray, the rights to the drug candidate compounds are shared between Toray and the company, and the company is to receive revenue in proportion as share of compounds



# Profitability potential of platform-type business

We have short-term (during drug discovery) and medium-term (during development) business revenue opportunities under the existing joint drug discovery research agreements. In the long term after the drug is launched, we expect to receive milestone revenue based on sales, as well as royalties of several percent not included in the figures below

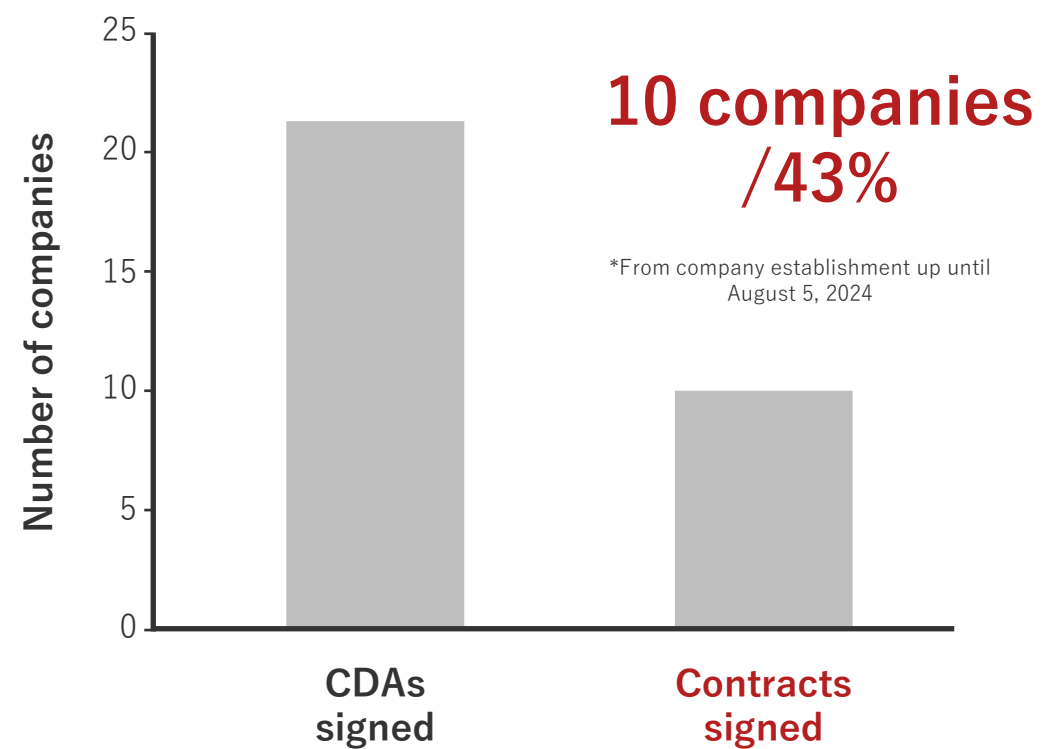




# Securing the number of CDAs needed to form new partnerships with 2 companies/year

Of the pharmaceutical companies that have signed confidentiality agreements (CDAs), the probability of reaching a contract as a result of negotiations under the CDA is 43%, and the median time between signing a CDA and reaching a contract is 14 months. In 2024, we expect to sign contracts with 2 companies based on the number of CDAs currently in effect. Each year after 2025, we aim to obtain a commensurate number of CDAs to sign contracts with 2 companies

Results: From CDA to contract signing\*



\*Since the end of June, one CDA has been newly added, bringing the total to five companies.

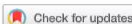


EXPERT OPINION ON DRUG DISCOVERY  
2024, VOL. 19, NO. 4, 415–431  
<https://doi.org/10.1080/17460441.2024.2313455>



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Veritas In Silico Inc, Tokyo, Japan

## ABSTRACT

**Areas covered:** The topics described in this review include the recent applications of AI in the identification of RNA targets, RNA structure determination, screening of chemical compound libraries, and hit-to-lead optimization. The impact and limitations of the recent AI applications are discussed, along with an outlook on the possible applications of next-generation AI tools for the discovery of novel RNA-targeted small molecule drugs.

**Expert opinion:** Key areas for improvement include developing AI tools for understanding RNA dynamics and RNA – small molecule interactions. High-quality and comprehensive data still need to be generated especially on the biological activity of small molecules that target RNAs.

## ARTICLE HISTORY

Received 31 October 2023  
Accepted 30 January 2024

## KEYWORDS

Artificial intelligence; deep learning; hit-to-lead optimization; machine learning; RNA structure; RNA-targeted small molecule drugs; target identification







# 2

## Financial Highlights



# Summary of Business results for the first half of FY2024

The joint drug discovery research with each pharmaceutical partner for mRNA-targeted small molecule drug discovery is progressing steadily  
Business revenue totaled 115 million yen, including research support funds and milestone payment

Summary of Profit and Loss Statements (millions of yen)		Breakdown (millions of yen)	
FY2024 (interim period)			
Business revenue	115	Research support funds	55
		Milestone revenue	60
Business expenses	182	R&D expenses	70
		SGA expenses	112
Operating loss	- 66		
Non-operating profit and loss	- 22	Listing expenses	12
		Share issuance costs	9
Ordinary loss	- 88		
Net loss	- 90		

\*Comparisons of financial statements between the first half of FY2024 and FY2023  
are omitted as quarterly financial statements, etc. were not prepared for FY2023



# Trends in Financial Position

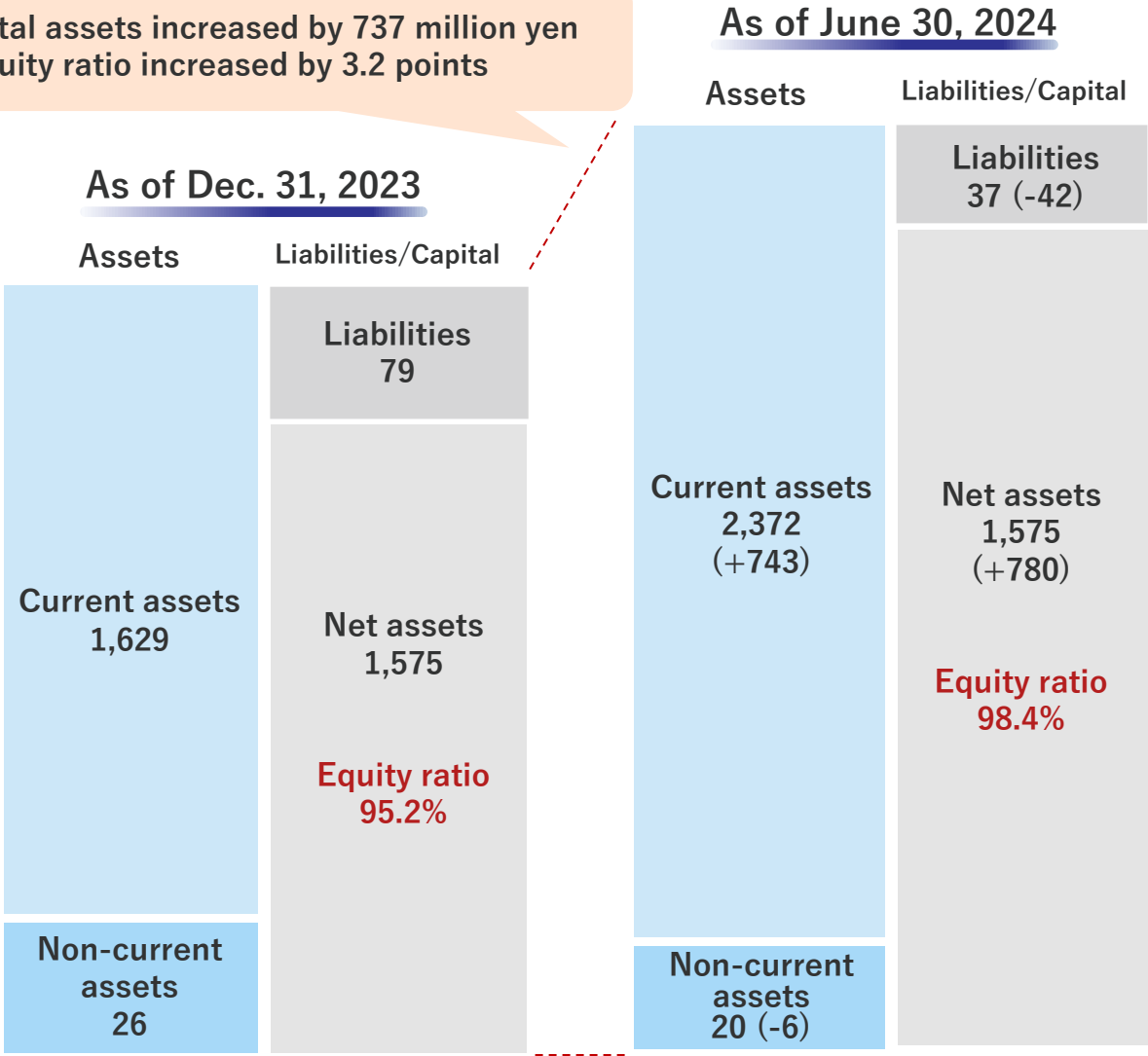
Current assets increased due to public offering at the time of TSE listing, etc., and equity ratio as well. The capital decreased due to capital reduction in April 2024

Balance Sheets (millions of yen)

	As of Dec. 31, 2023	As of June 30, 2024
Cash and deposits	1,549	2,256
Total current assets	1,629	2,372
Property, plant and equipment	23	18
Total non-current assets	26	20
Total assets	1,655	2,393
Total liabilities	79	37
Share capital	90	77
Total net assets	1,575	2,355
Total liabilities and net assets	1,655	2,393

## Balance Sheets Transition

Total assets increased by 737 million yen  
Equity ratio increased by 3.2 points





# Status of Cash flows

Increase in cash and deposits due to public offering at the time of TSE listing, etc.

## Condensed Interim Statements of Cash Flows (millions of yen)

January-June 2024	
Cash and cash equivalents at beginning of period	1,549
Cash flows from operating activities	- 139
Cash flows from investing activities	- 1
Cash flows from financing activities	848
Net increase (decrease) in cash and cash equivalents	707
Cash and cash equivalents at end of period	2,256

Breakdown	(millions of yen)
Interim loss before income taxes and equity in earnings	- 88
Increase in trade receivables	- 23
Decrease in advances received	- 12
Increase in advance payments	- 11
Acquisition of property, plant and equipment	- 1
Stock issuance	861
Listing-related expenses	- 12





# 3 Business Overview



# Biotech with extensive research capabilities on mRNA-targeted small molecule drug discovery

Name	Veritas In Silico Inc. (VIS)
Established	November 17, 2016
Main office	1-11-1 Nishigotanda, Shinagawa-ku, Tokyo 141-0031, Japan
Research facilities	Basic Research Facility: Kanagawa, Japan Applied Research Facility: Niigata, Japan
CEO	Shingo Nakamura
Employees	14 (As of June 2024)
Capital	77 million yen (As of June 2024)
Business description	Creating mRNA-targeted small molecule drugs through joint drug discovery research with pharmaceutical companies using our proprietary drug discovery platform, ibVIS®



Basic Research Facility

(At the Kawasaki Business Incubation Center)



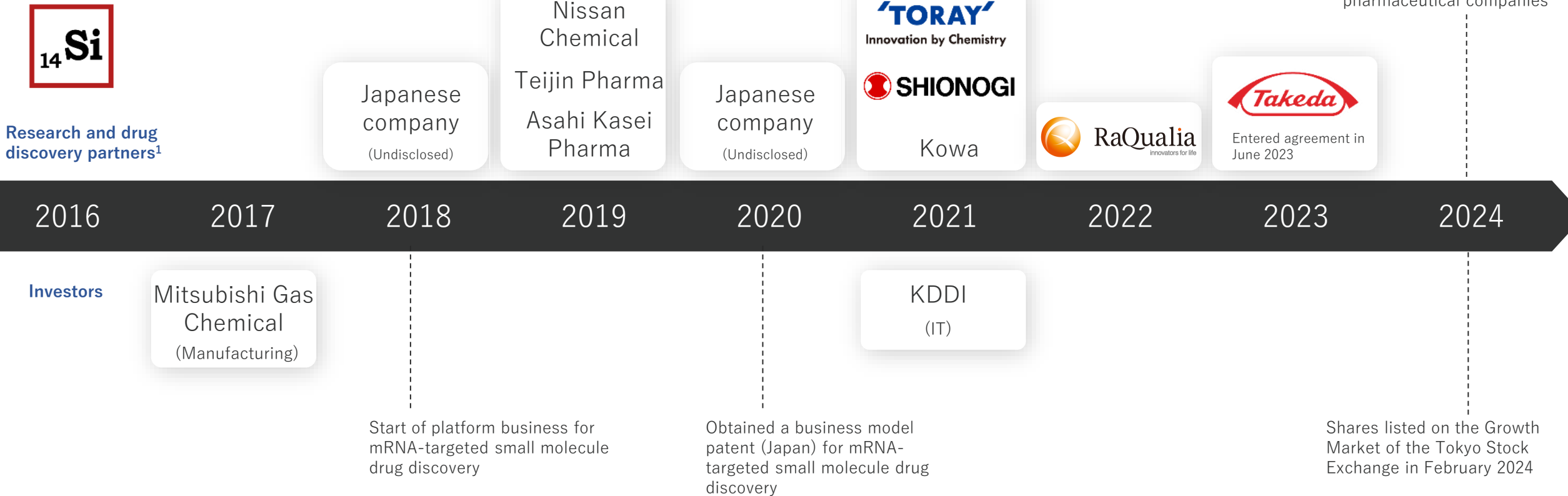
Applied Research Facility

(At the Niigata University of Pharmacy and Medical and Life Sciences)



# Steadily expanding business by earning the trust of renowned companies

We are advancing an innovative approach—mRNA-targeted small molecule drug discovery—with support from our business partners and together with our research and drug discovery partners



(注) 現在、東レ、塩野義製薬、ラクオリア創薬、武田薬品の4社と共同創薬研究を実施中

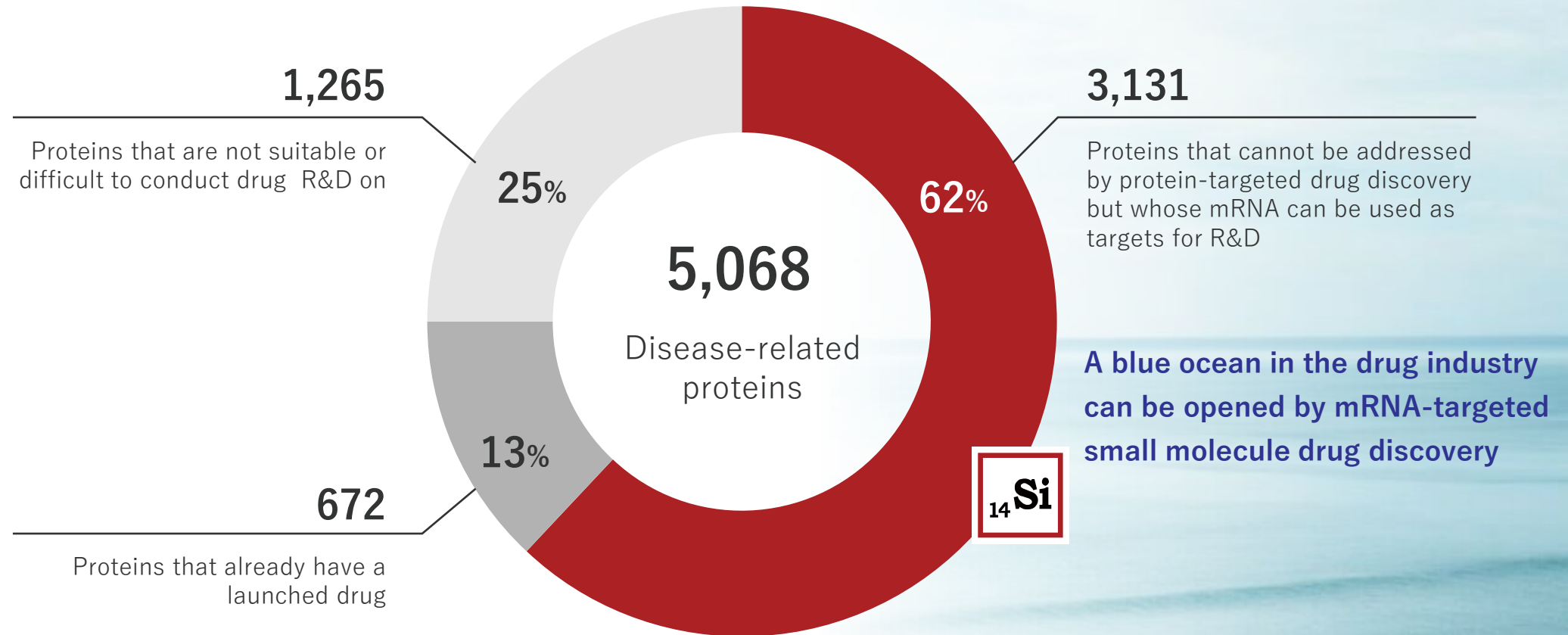
<sup>1</sup>Currently conducting joint drug discovery research with four companies: Toray, Shionogi, RaQualia Pharma, and Takeda

<sup>2</sup>As of June 2024



# The “undruggable” into the “druggable” with mRNA-targeted small molecule drug discovery

Disease that were considered “undruggable” by conventional protein-targeted drug discovery technologies can become “druggable” by targeting the mRNA

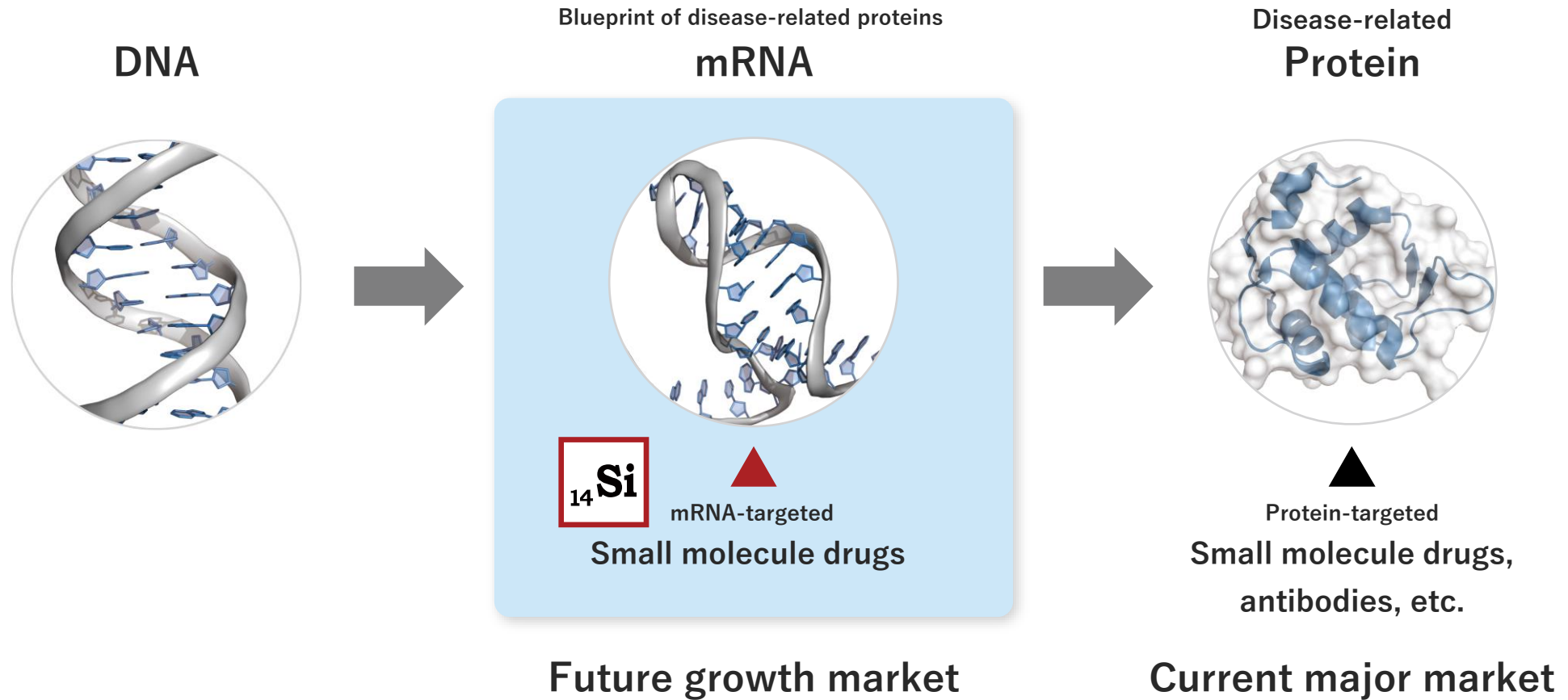


Source : Based on the Human Protein Atlas, DrugBank, KS analysis, 2018



## Opening a blue ocean by creating mRNA-targeted small molecule drugs

We aim to target the mRNA—the blueprint of proteins—with small molecule drugs, which are desirable because they can be administered orally and are economical. Future market growth is expected with this new blue ocean strategy as it can tackle the problems related to small molecule drug discovery



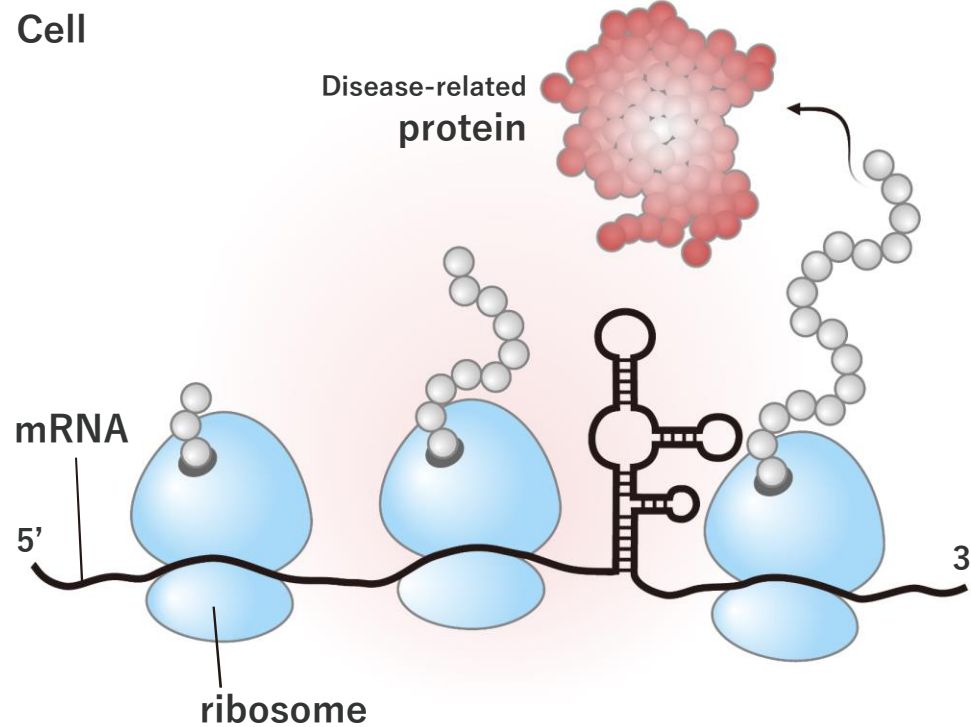
Note: Research and development on mRNA-targeted small molecule drugs is still mostly in the drug discovery stage even outside Japan. No small molecule drugs have been launched due to this drug discovery approach as of June 2024.



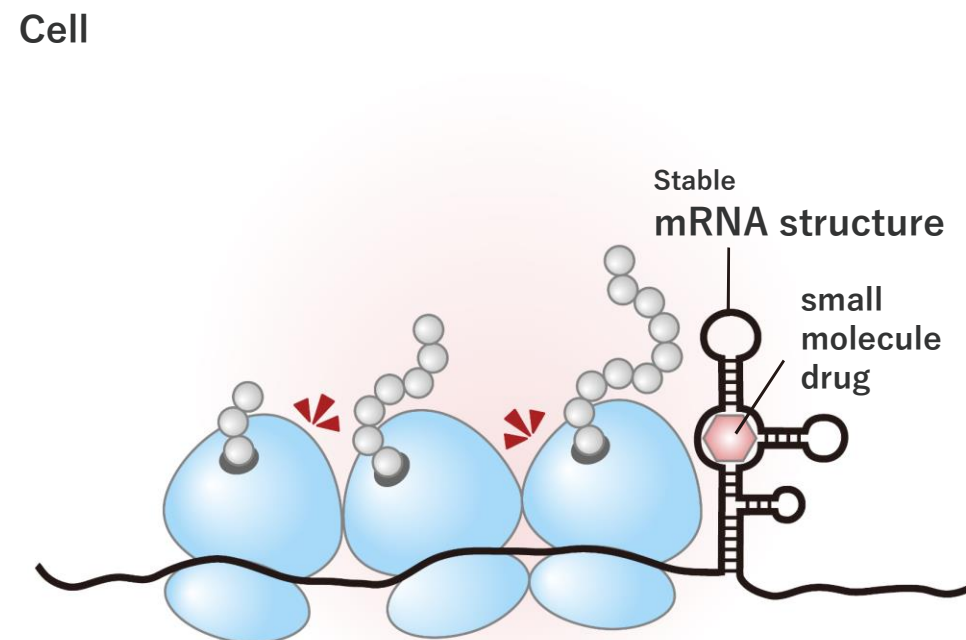
## Versatile mechanism of action applicable to the treatment of various diseases

In the cell, proteins are synthesized by the ribosome by reading information on the mRNA from left to right. When the structure on the mRNA is stabilized by a small molecule drug, the ribosome is unable to read the mRNA information and protein synthesis stops

Without small molecule drugs



With small molecule drugs

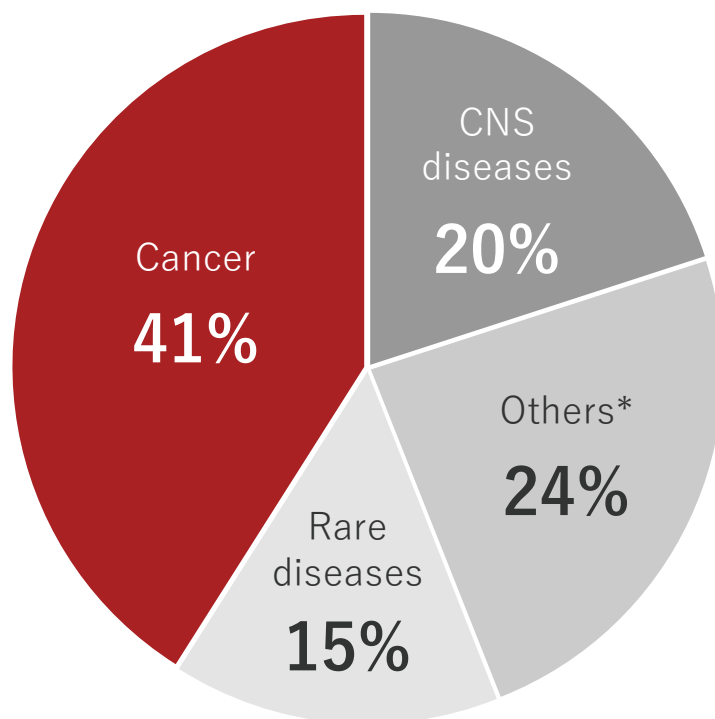




## Addressing unmet medical needs with ibVIS®

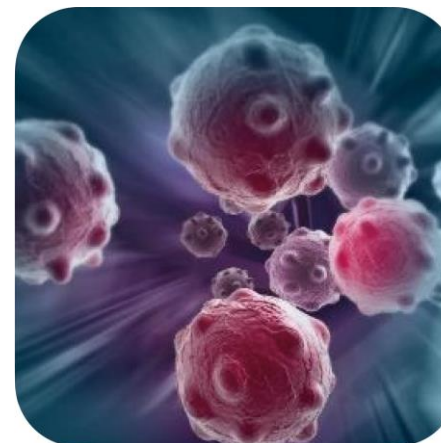
We have applied our drug discovery platform, ibVIS®, to drug discovery against 100 genes of interest (GOIs), which cover a wide variety of disease areas that have been disclosed by pharmaceutical companies. Even from the perspective of drug discovery experts, ibVIS® is applicable to a wide variety of diseases. Based on the GOIs, cancer is the largest disease area of focus, followed by central nervous system (CNS) diseases

### Disease areas revealed by the GOIs



\*Includes cardiovascular disease, immune disorders, infectious diseases, etc.

Based on GOIs disclosed by pharmaceutical companies as of June 30, 2024



### Cancer

The mechanisms of cancer are diverse, and there are many cancers that cannot be treated with conventional drug discovery. Since the number of patients is large, it is desirable to develop new small molecule drugs that can be supplied in large quantities



### CNS diseases

The blood-brain barrier (BBB) in the brain (central nervous system) is a protective system that blocks substances that affect nerve cells. Small molecule drugs that can cross the BBB would be effective in treating CNS diseases





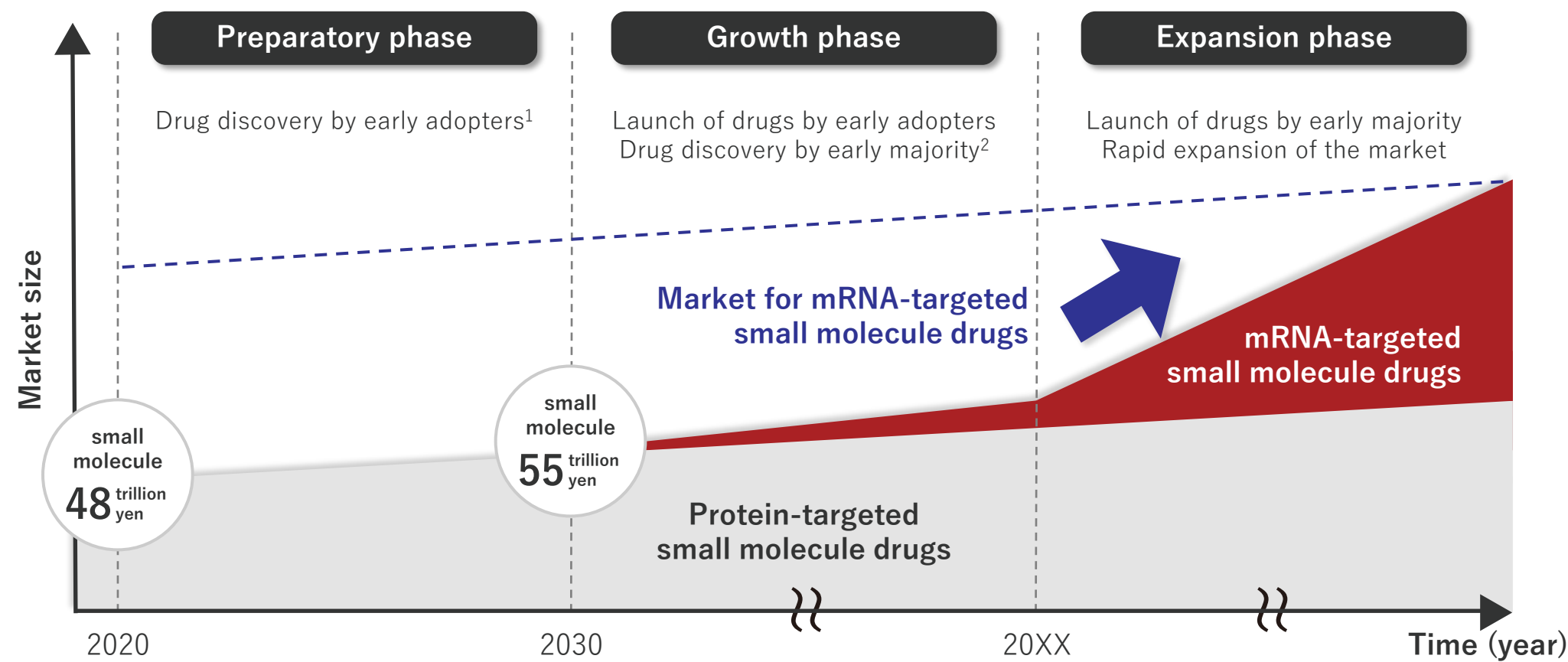
# 4

## Business Model



# Market for mRNA-targeted small molecule drugs to rival that of protein-targeted drugs

At present, mRNA-targeted small molecule drugs are in the **preparatory phase**. Once mRNA-targeted small molecule drugs are launched in the market (**growth phase**), they are expected to spread rapidly to "undruggable" diseases (**expansion phase**) and become a large drug market in the future



<sup>1</sup>People who buy products and services after the innovators  
<sup>2</sup>People who are influenced by early adopters

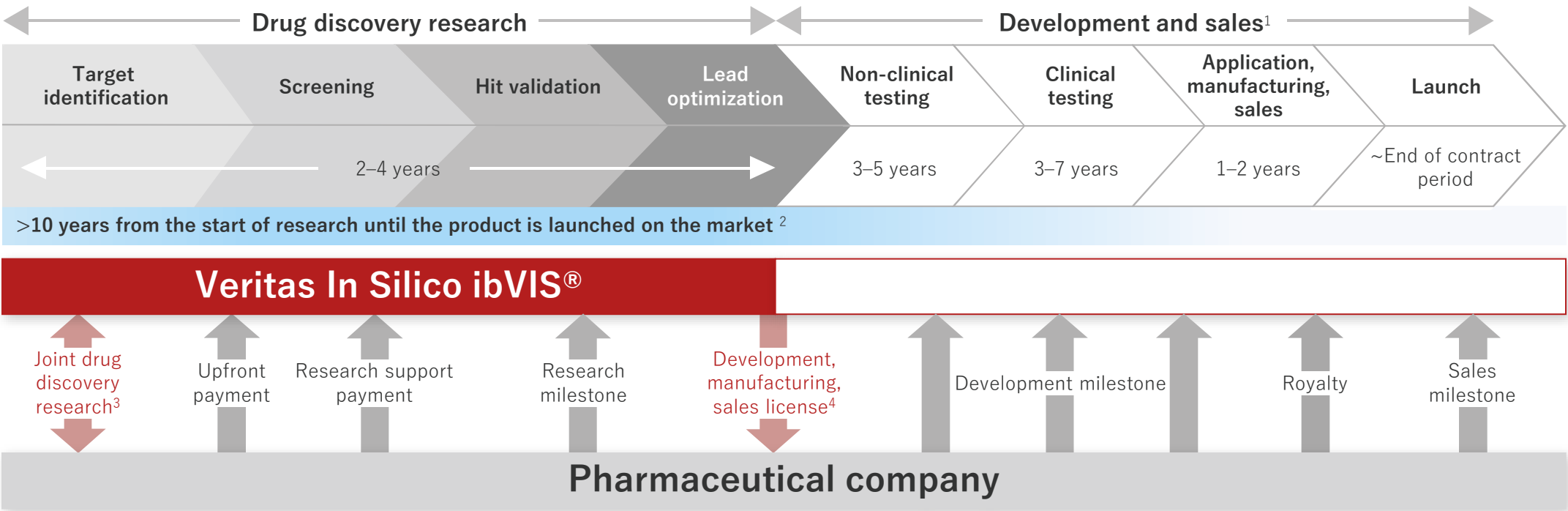
Source: Based on data from the Japanese Prime Minister Office website

\*Estimates made by the company



# Stable business revenue from the early stages of drug discovery over the long term

Under the joint drug discovery research agreement between our company and pharmaceutical companies, we will receive business revenue in exchange for the right to use our drug discovery platform, ibVIS®. After licensing the rights to develop, manufacture, and sell the drug, we will continue to receive revenue based on our contribution to the drug discovery research



Different business revenues received or to be received	
Upfront payment	Payment at the time of contract signing
Research support payment	Per target; As compensation for conducting research
Milestone	Upon achievement of pre-determined events based on R&D and sales progress
Royalty	Based on annual sales after the start of the drug launch

<sup>1</sup>At present (as of June 30, 2024), no partner has progressed to the development and sales step.  
<sup>2</sup>The average duration is based on the typical duration of drug discovery R&D, and the actual R&D durations may differ significantly from those given here.  
<sup>3</sup>The use of ibVIS® is limited to the duration of the joint drug discovery research between our company and pharmaceutical companies.  
<sup>4</sup>In some cases, arrangements (compensations) for development, manufacturing, and sales licenses may be included at the time of the initial joint drug discovery research agreement.



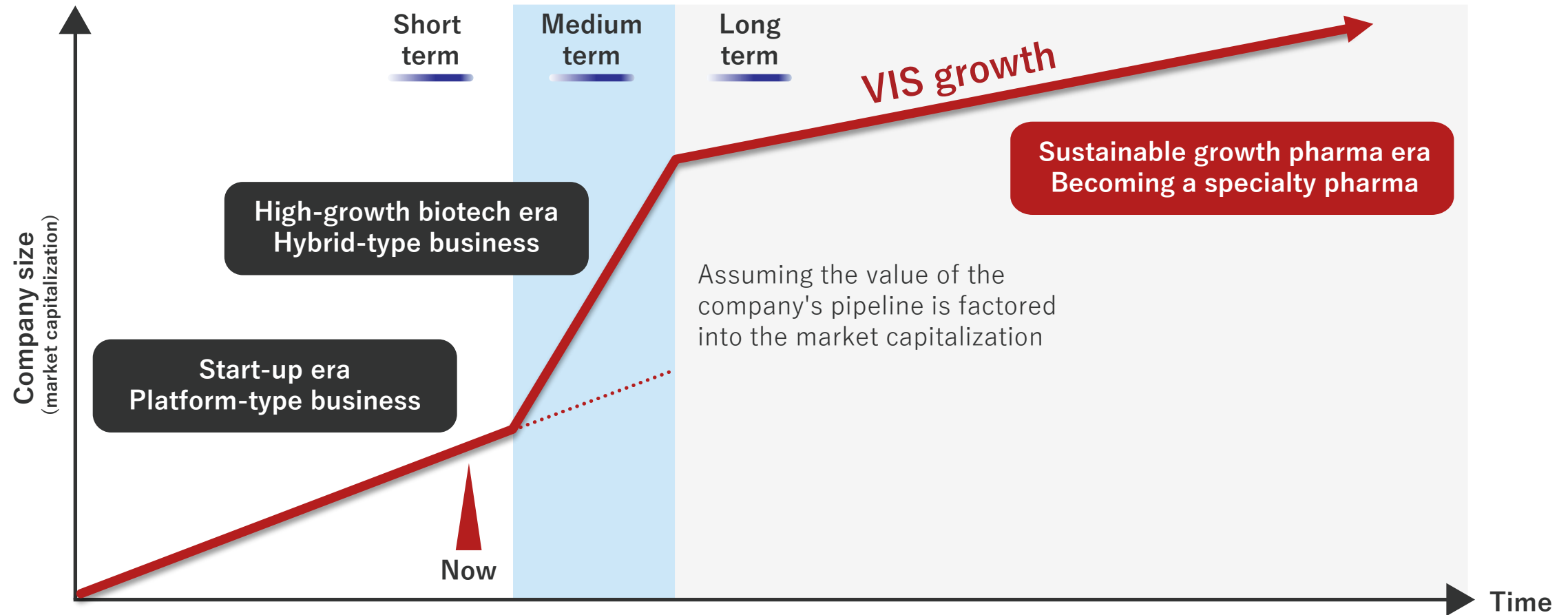


# 5 Growth Strategy



## From start-up biotech company to specialty pharma with sustainable growth

We are currently shifting from a platform-type business to a hybrid-type business that can achieve a high growth rate. Ultimately, for sustainable growth, we intend to transition to a pharmaceutical company (specialty pharmaceutical) equipped with capabilities to conduct research, development, and sales of drugs, with a focus on mRNA-related drug discovery.

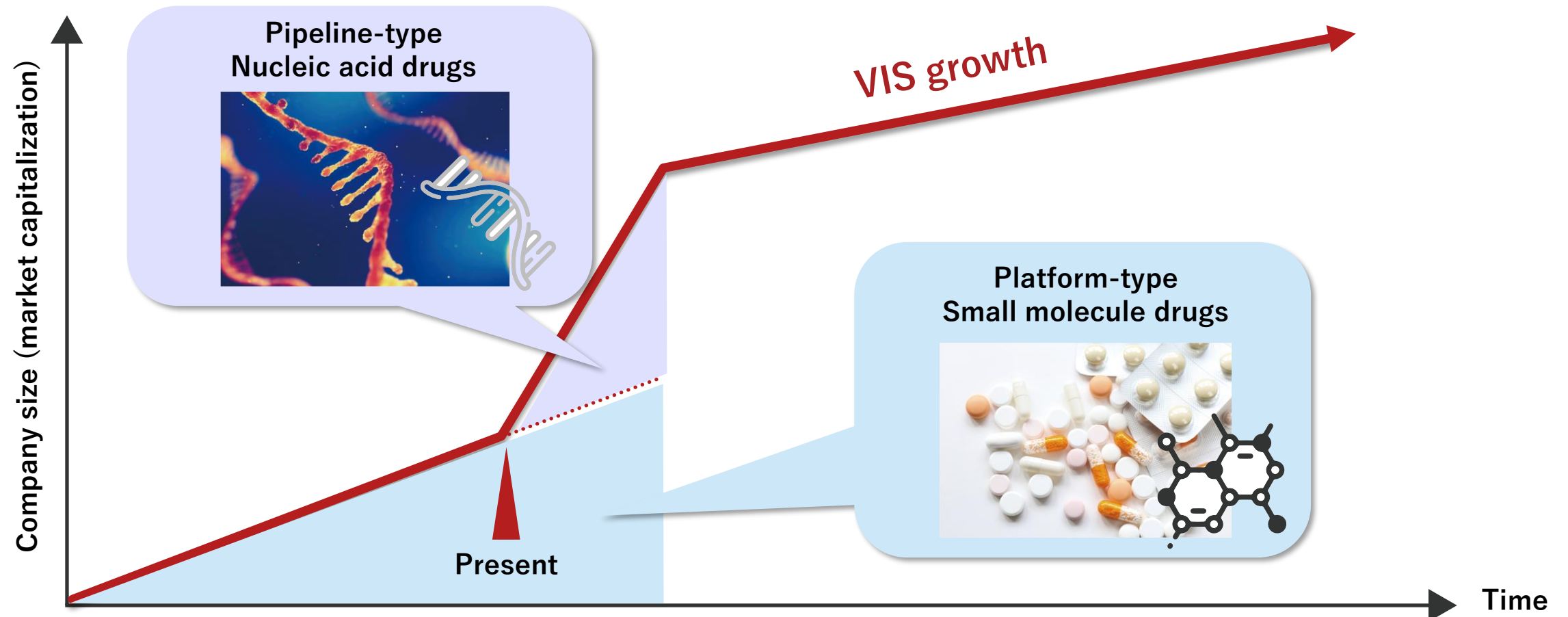


Note: This is only an image of the growth curve we aim to achieve



## Conversion to a hybrid-type business as a prelude to specialty pharma.

We aim to create our own pipeline with a focus on nucleic acid drugs while expanding our platform-type business of mRNA-targeted small molecule drugs



\*This is only an image of our targeted growth and does not imply real market capitalization trends



## Sustainable growth through diversification of nucleic acid-related drug discovery business

Our in silico RNA structural analysis can be applied to various nucleic acid-related drug discovery approaches. When we shift to the hybrid-type business, nucleic acid drugs are the leading candidates for our pipeline. In addition, we will diversify our business in the future by making mRNA drugs and ncRNA-targeted drugs a part of our businesses through joint research with academia and companies

← **Short term/Platform** →   ← **Medium term/Pipeline** →   ← **Long term business development through joint research** →

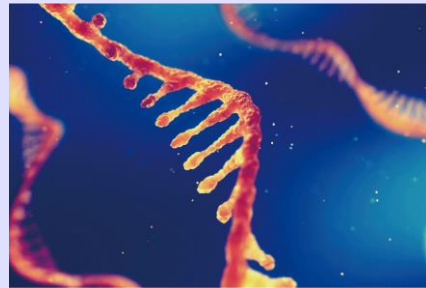
01



### mRNA-targeted small molecule drugs

Provide solutions to medical needs that cannot be addressed by protein-targeted small molecule drug discovery or that can only be addressed by expensive therapies, such as antibodies

02



### Nucleic acid drugs (mRNA-targeted)

Create simpler nucleic acid drugs with fewer side effects and high cell membrane permeability that will be a viable solution to rare disease treatment needs

03



### mRNA drugs

Design mRNA sequences for medical use, an alternative to protein replacement therapy and a solution to therapeutic needs

04



### ncRNA-targeted drugs

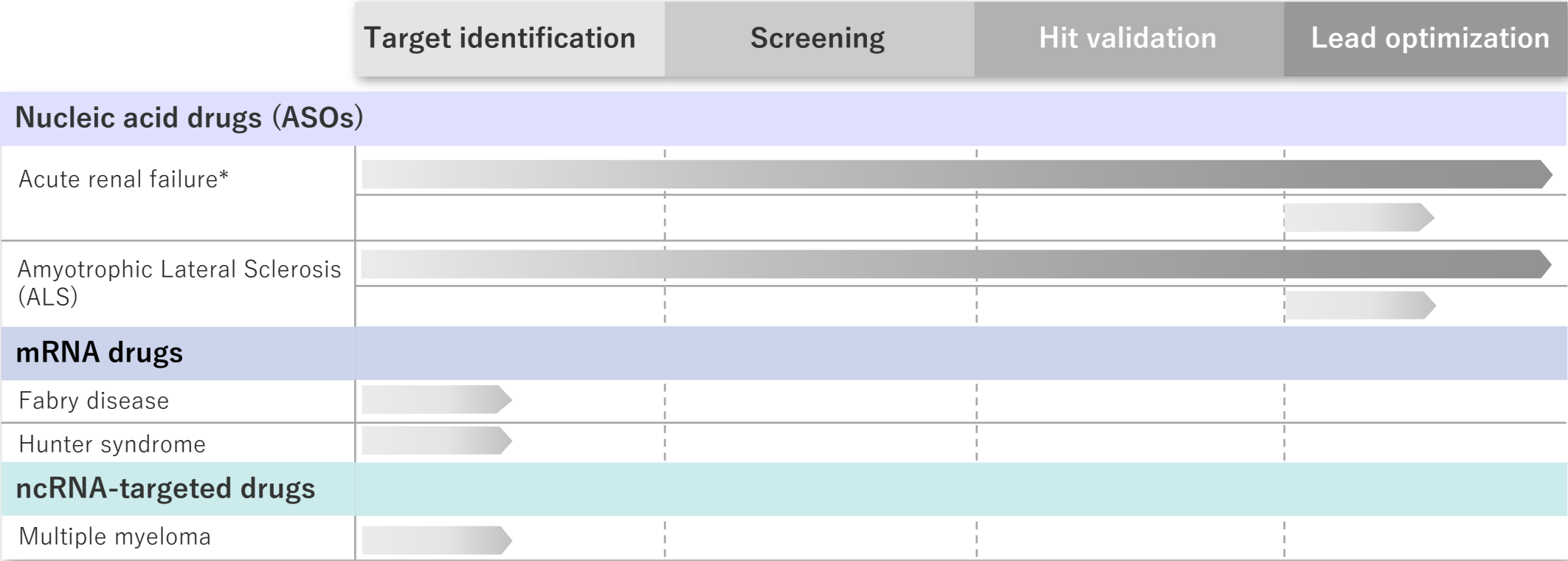
Generate small molecule and nucleic acid drugs that regulate non-coding RNAs (ncRNAs), which do not serve as the blueprint for proteins



In-house pipeline candidates, etc.

# Nucleic acid drug pipeline candidates and other projects to support business stability

We have pipeline candidates of nucleic acid drugs, mainly for rare diseases, which are the basis of a hybrid-type business. Nucleic acid drugs have a short drug discovery research period of only 8 months at our company, so new projects are also promising in-house pipeline candidates. The projects for mRNA drugs and ncRNA-targeted drugs are currently in the basic research (target identification) stage.



\*WO2021/002359 Nucleic acid drug and use thereof



## Aiming to conclude contracts with ODS clients that could not be approached before

ODS hosted a webinar in March 2024 to educate on mRNA-targeted small molecule drug discovery. This led to a CDA with a European pharmaceutical company and contract negotiations are underway. ODS is our strong partner in negotiating contracts with pharmaceutical companies and so on, as we expand into Europe.

会社名	Oncodesign Services (ODS)
代表者	Fabrice Viviani, President and CEO
設立年	1995年
社員数	230名 (PhD取得者 22%)
顧客数	1,000
WEB	<a href="https://www.oncodesign-services.com/">https://www.oncodesign-services.com/</a>

2024年6月末現在

出典: Oncodesign Services社のウェブサイト





## Management that takes into consideration social and environmental sustainability

We will contribute to the health and welfare of as many patients as possible through sincere efforts to create small molecule drugs in collaboration with pharmaceutical companies and academia. We intend to practice management that considers social and environmental sustainability by improving the capabilities of society's society and technology and fostering a corporate culture that is rewarding to work in

### Initiatives related to our business activities

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To realize a warm society filled with hope

- ◆ Meeting unmet medical needs with novel drug discovery technologies
- ◆ Working with drug discovery partners to create innovative medicines

### Efforts to build a business foundation

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- ◆ Secure and develop excellent human resources
- ◆ Foster a rewarding corporate culture
- ◆ Diversify human resources and create an organization that makes the most of each individual
- ◆ Provide a pleasant work environment (promoting the use of annual paid leave)
- ◆ Management and promotion of employee health
- ◆ Purchase following the Green Purchasing Law

### Realization of a Sustainable Society Initiatives for Sustainability in Science and Technology

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- ◆ Enhancement of our drug discovery capabilities through collaborative research with academia on mRNA
- ◆ Contribution to academia through lectures and presentations at universities and other educational institutions



## Efforts Toward Sustainability of Science and Technology Capability in Society

Through collaborative research with academia, lectures, and presentations, we will improve our drug discovery technology and contribute to the improvement of science and technology in Japan and, by extension, the realization of a sustainable society.

### Collaboration with academia on mRNA

Osaka University 2 projects

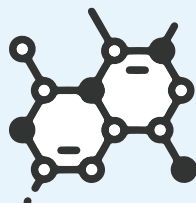
Chiba Institute of  
Technology

Sophia University

Tokyo University of  
Agriculture and Technology

Konan University

**mRNA-targeted small  
molecule drugs**



Niigata University of Pharmacy  
and Medical and Life Sciences

The Jikei University School  
of Medicine

Stanford University



**Nucleic acid drugs**

### Lectures and presentations at educational institutions

#### Lectures conducted annually

Tokyo Institute of Technology

Chiba Institute of Technology

#### Presentations conducted in 2024

Nikkei Business Forum

The Society of Synthetic Organic  
Chemistry, Japan

mRNA Targeted Drug Discovery  
Research Organization

Frontiers of Drug Discovery  
by LUNK-J





## Mission

**Build a warm society where every patient can look forward to a brighter future through the realization of mRNA-targeted drugs**

We, as pathfinders pioneering the frontiers of drug discovery, ...

Aim to provide the best treatment for any disease





**Veritas In Silico**