



Veritas In Silico

Bringing new hope with mRNA-targeted drugs

This document has been translated from the Japanese original for reference purposes only. In case of discrepancy, the Japanese original shall prevail.

Company Presentation

Veritas In Silico Inc.

TSE Ticker code: 130A

February 13, 2025



Future Growth Strategy

Advancing platform business for mRNA-targeted small molecule drug discovery

We are supposed to acquire 2 new contracts each year* to expand the platform business using the drug discovery platform, ibVIS®, and progress joint drug discovery research not only with 4 pharmaceutical companies currently underway but also with new customers

*The target number of contract conclusions for FY2025 is 4 contracts in total, 2 new contracts in addition to another 2 contracts that were scheduled in FY2024
One of the 2 new contracts was signed with LCC ahead of schedule in December 2024.

Creating in-house pipelines for the transition to hybrid business

We aim to create one in-house pipeline annually, primarily nucleic acid drugs, leveraging drug discovery technology honed through our platform business. We strive to enhance our shareholder value from a business perspective

Transforming into specialty pharma after growing through a hybrid business model

We will balance revenue-generating platform business with investment-focused pipeline creation, which allows us to continuously enhance shareholder value and ultimately establish ourselves as a specialty pharmaceutical company after our medium-term management plan.



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1 Corporate Overview



Mission

Build a 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

Biotech with extensive research capabilities on mRNA-targeted small molecule and nucleic acid 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	Shinkawasaki Research Institute: Kanagawa, Japan Niigata Research Institute: Niigata, Japan
CEO	Shingo Nakamura
Employees	19 (As of December 31, 2024)
Capital	77,175 thousand yen (As of December 31, 2024)
Business description	Creating mRNA-targeted small molecule drugs through joint drug discovery research with pharmaceutical companies using our proprietary drug discovery platform, ibVIS®, as well as developing its pipelines from 2025



Shinkawasaki Research Institute

(At the Kawasaki Business Incubation Center)



Niigata Research Institute

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

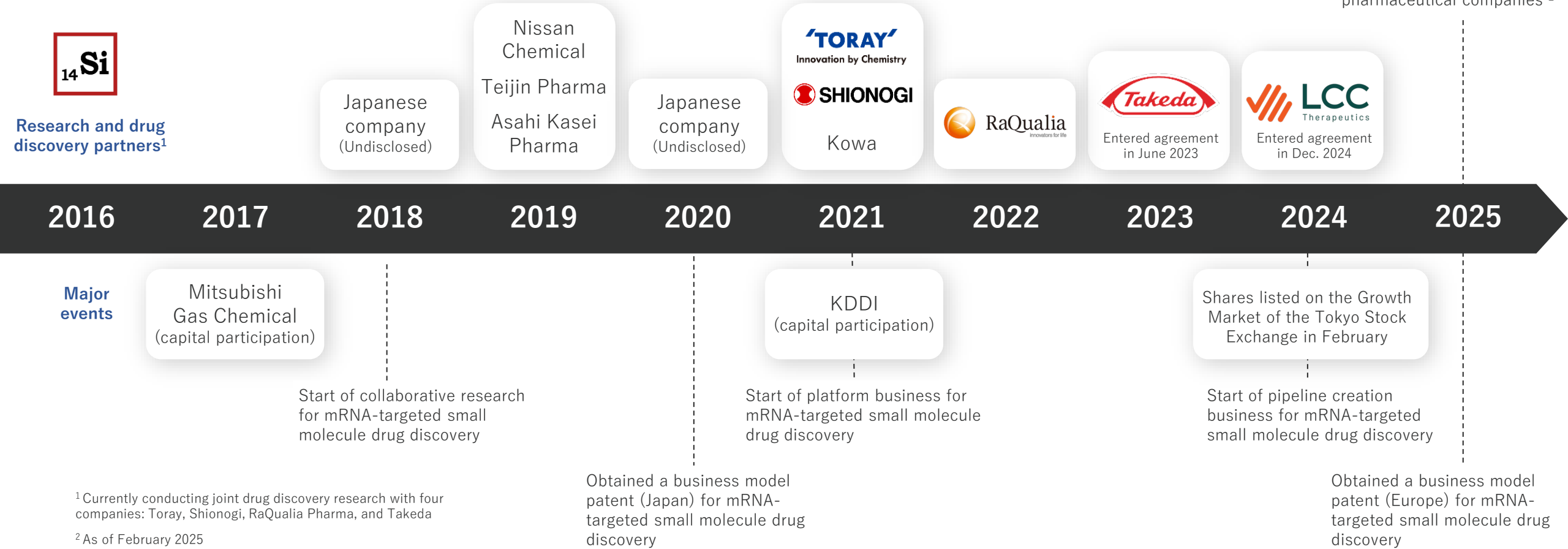
Steadily expanding business by earning the trust of renowned companies

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



Research and drug discovery partners¹

Negotiating contracts with domestic and foreign pharmaceutical companies ²



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

² As of February 2025

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

After the company's establishment, Shingo 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, Founder 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.

2016~ Management career

Representative Director and CEO of Veritas In Silico (current position)

*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

Management team with extensive operational experience

The management team, with extensive practical experience in the pharmaceutical and biotech industries, along with audit and supervisory board members with a wide range of experience in overseeing management, are working together to drive the business forward



Director
Hiroaki Hagiwara

Over 30 years of work experience as an administrator in a wide range of industries and has completed two IPOs, including Veritas In Silico



Director
Isao Koda, PhD

More than 40 years of experience in the pharmaceutical industry, including approx. 15 years in the U.S., ranging from drug discovery research and clinical development to business development



Director
Kinichiro Kominami, PhD

Representative Director of Tech & FinStrategy, Inc.

A broad network of contacts in the biotechnology industry through working as a bioanalyst at Nomura Securities and others



Audit & Supervisory Board Member
Sadao Suzuki

Firsthand experience in asset management at a life insurance company and a wealth of audit and supervisory experience



Audit & Supervisory Board Member
Minoru Hirooka

Head of Hirooka Certified Public Accountant Office
Certified Public Accountant and Tax Accountant

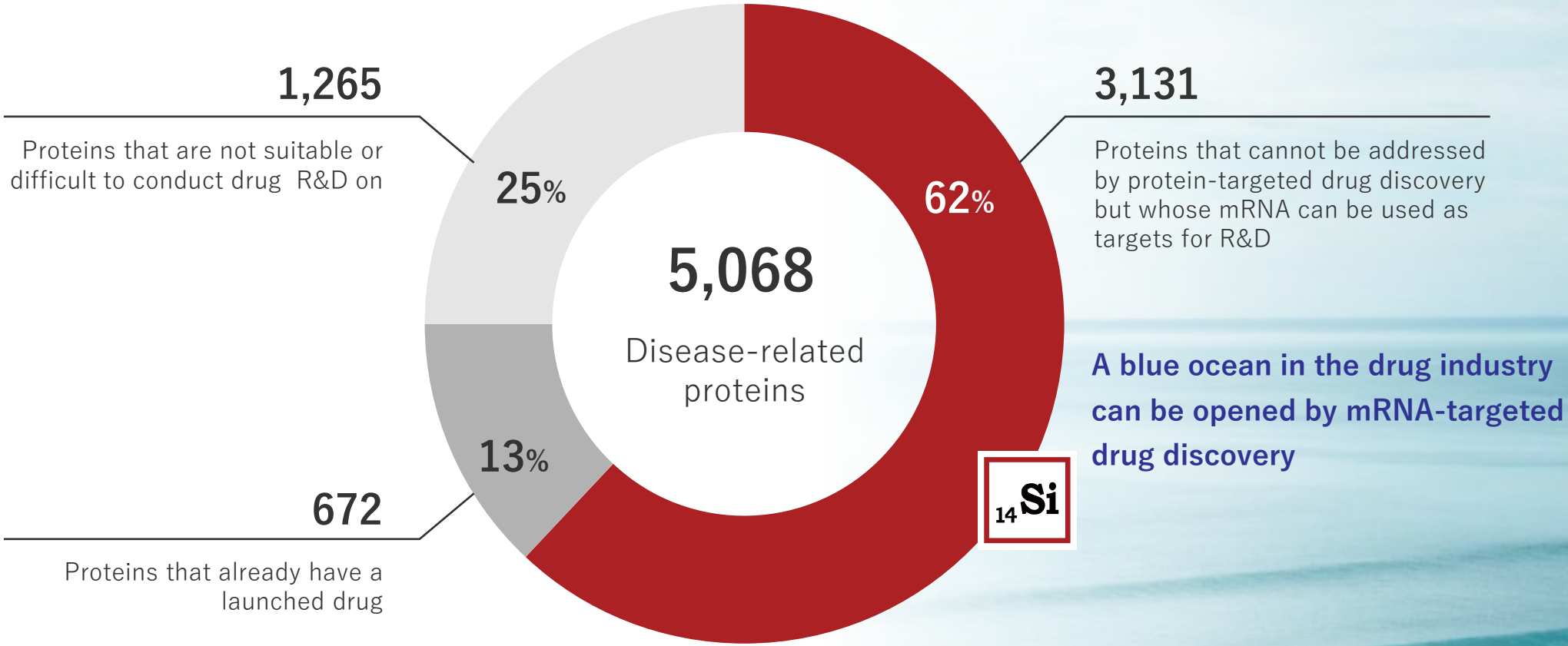


Audit & Supervisory Board Member
Minako Wakabayashi

Orrick, Herrington & Sutcliffe LLP/
Orrick Tokyo Law office
Representative of Tokyo office,
Foreign Law Joint Enterprise
Attorney (Managing partner)

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

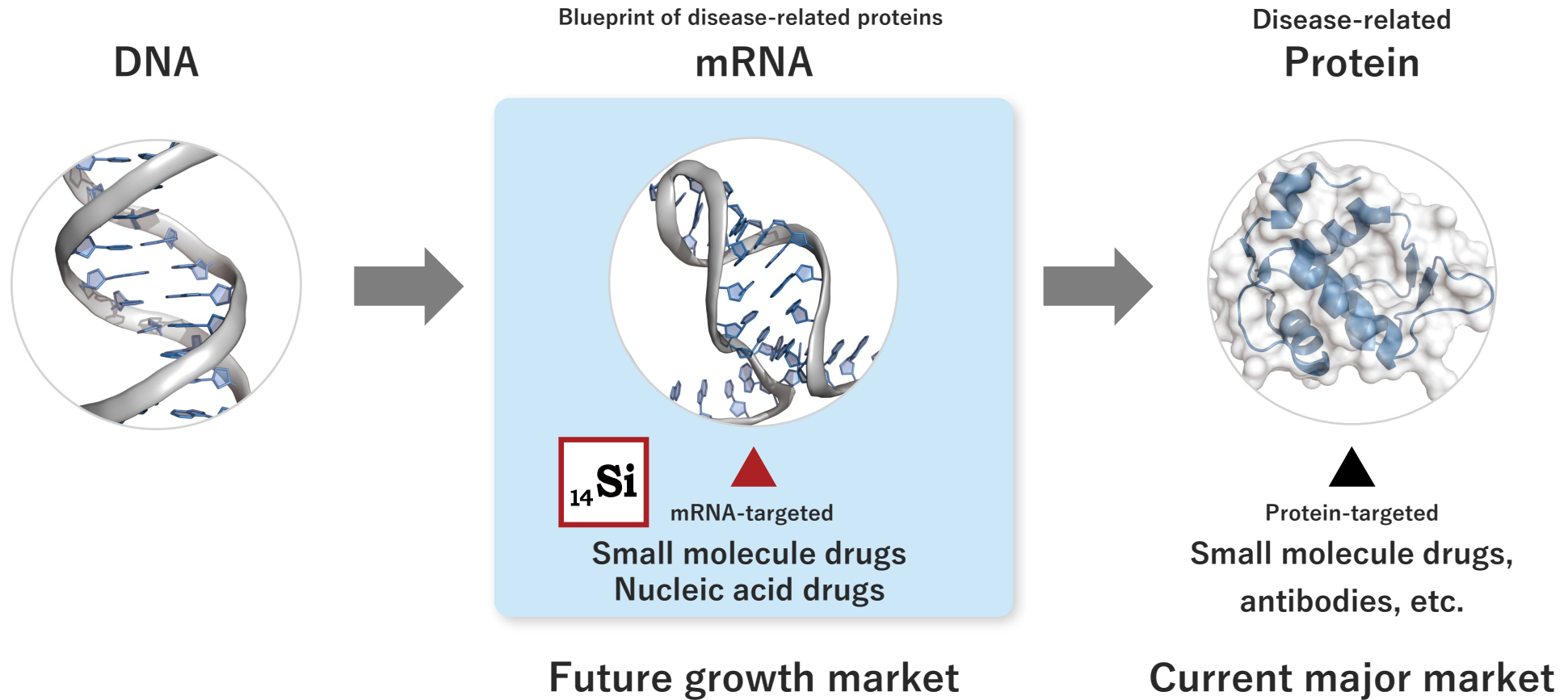
Diseases that were considered “undruggable” by conventional protein-targeted drug discovery technologies can become “druggable” by targeting the mRNA, There is potential to address unmet medical needs (medical needs for diseases with no effective treatments), which account for more than half of disease-related proteins



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

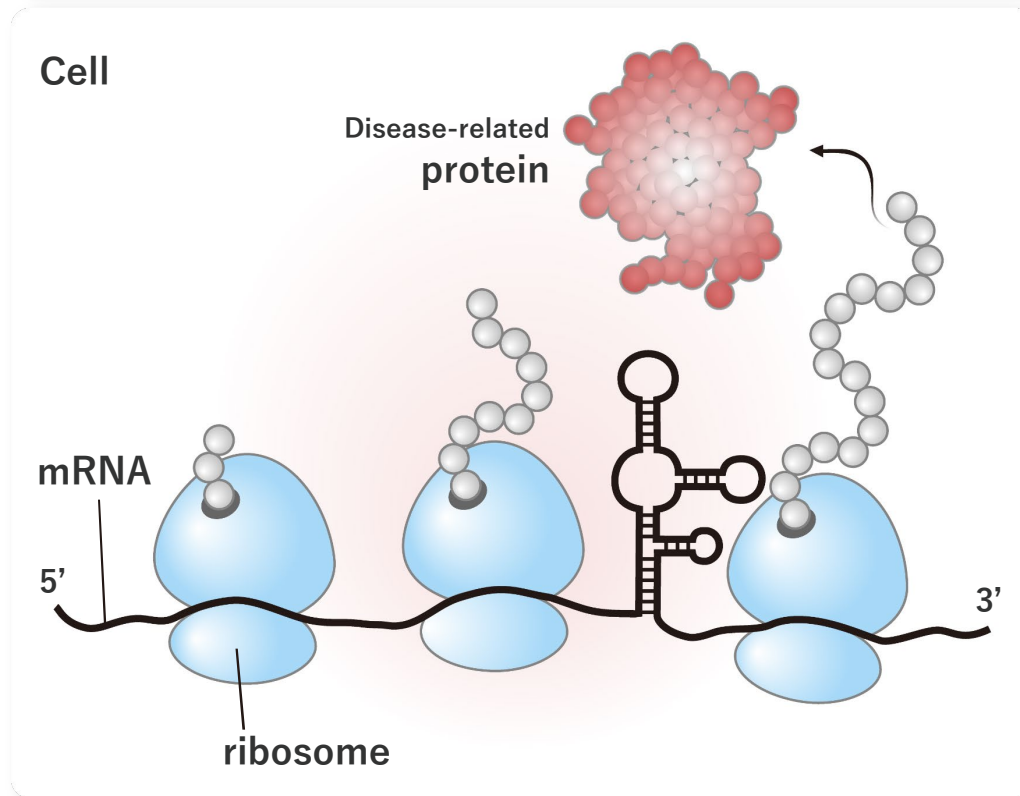


Note: Research and development on mRNA-targeted small molecule drugs is still mostly in the drug discovery stage even outside Japan. No small molecule drugs have been launched due to this drug discovery approach as of Dec. 31, 2024.

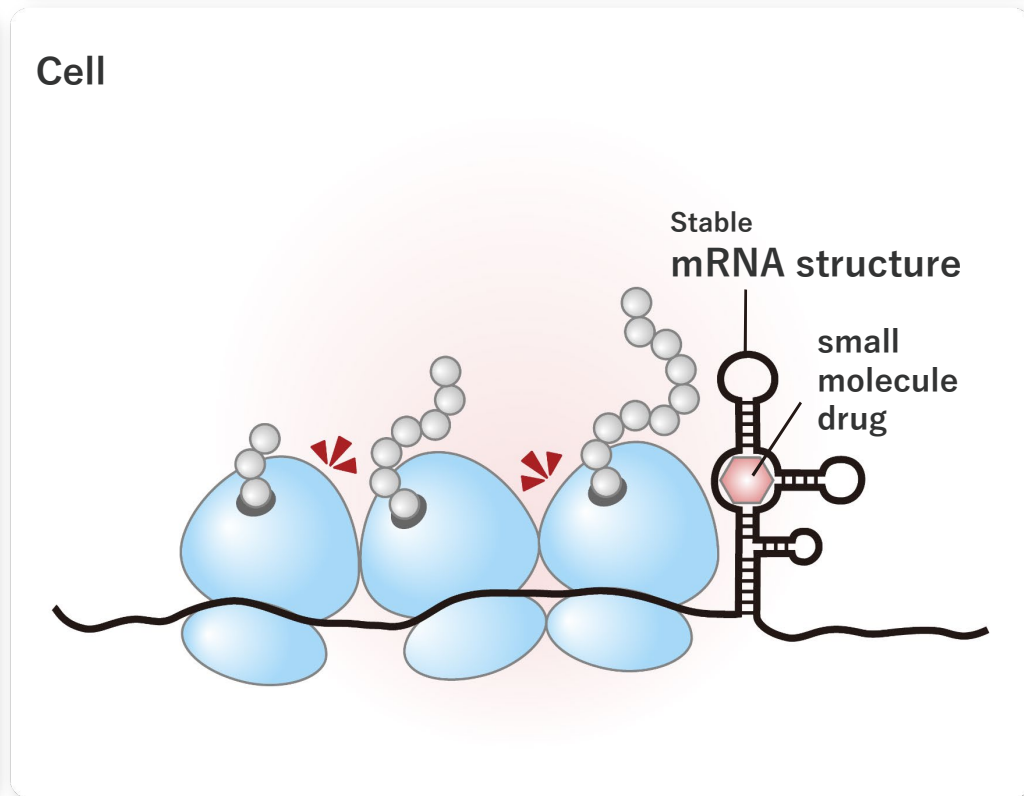
Versatile mechanism of action applicable to the treatment of various diseases

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



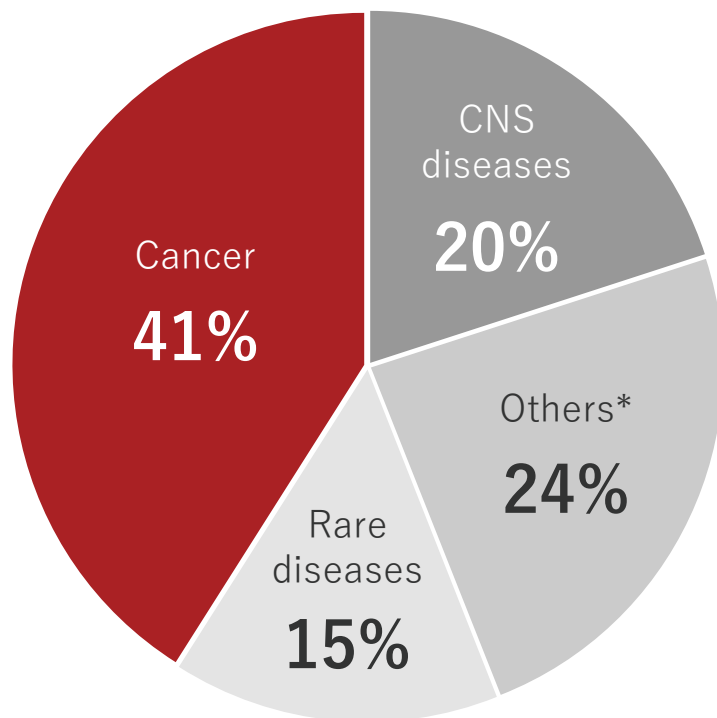
With small molecule drugs



Addressing unmet medical needs with ibVIS®

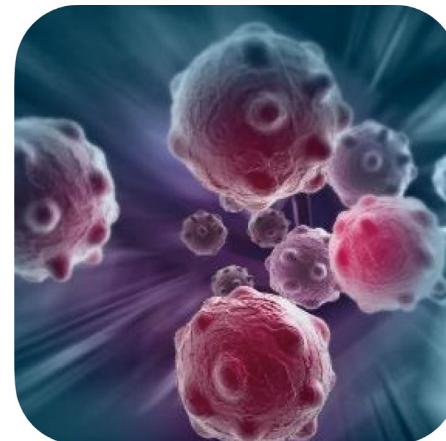
The pharmaceutical companies to which we have provided the information on our drug discovery platform, ibVIS®, have disclosed more than 100 genes of interest (GOIs) to us, and the disease areas of these GOIs are diverse. This implies that ibVIS® is applicable to a wide variety of diseases even from the perspective of drug discovery experts. 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 Dec. 31, 2024



Cancer

Cancer-causing genetic mutations are diverse, and many cancers cannot be treated with conventional protein-targeted 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

In the brain (central nervous system), the blood-brain barrier (BBB) blocks antibody drugs and other large molecule drugs

Small molecule drugs that can cross the BBB would be effective in treating CNS diseases

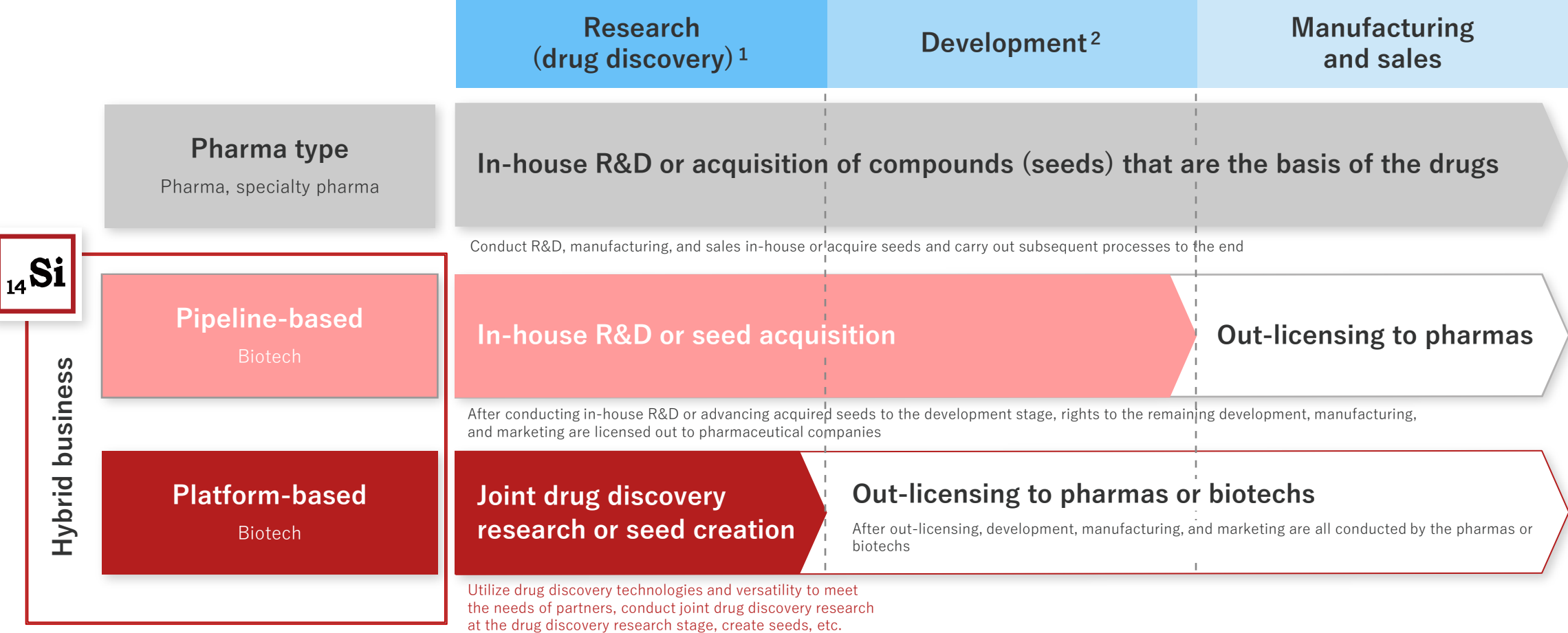


2

Business Model

Shift from platform business to hybrid business, leveraging platform technology

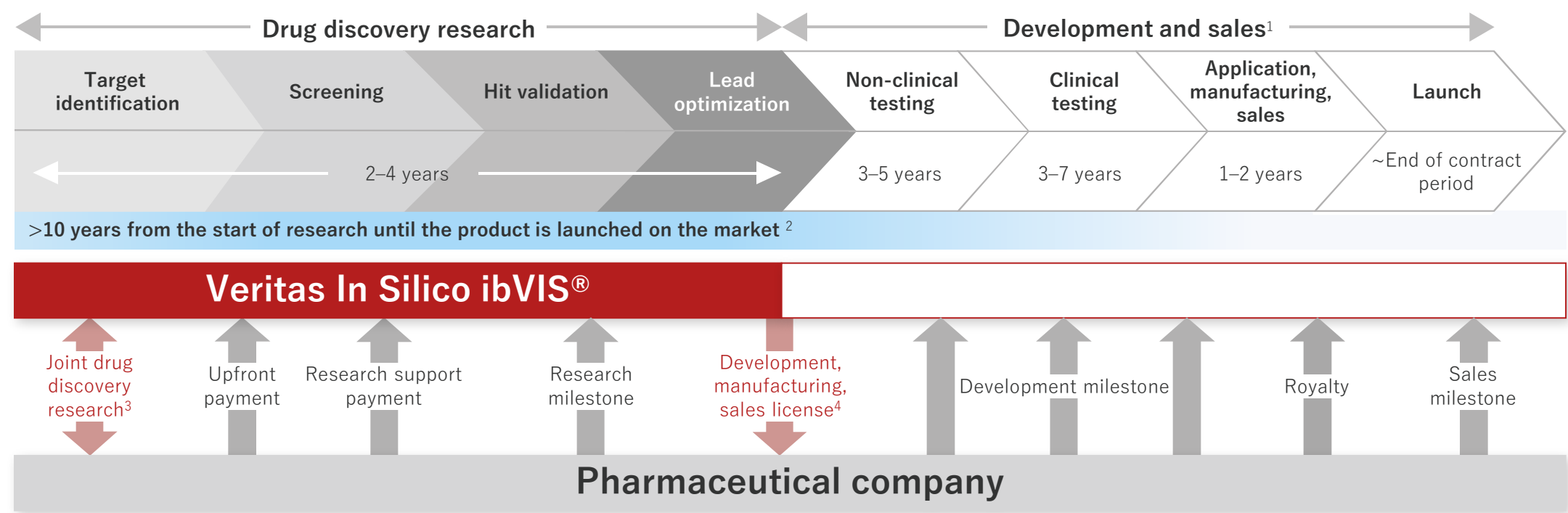
We focus on a platform-based business, leveraging our technological capabilities in mRNA-targeted small molecule drug discovery to meet a wide range of needs. Utilizing the expertise developed through our platform-based business, we have commenced expanding into a hybrid business to create our own pipeline



¹Drug discovery research is the stage of creating drug candidates that demonstrate sufficient efficacy and safety as pharmaceutical products.
²Development is the stage of proving the efficacy and safety of a drug candidate obtained through drug discovery research to regulatory authorities.

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®. This platform-based agreement allows us to receive revenue based on our contribution to the drug discovery research even after licensing the rights to develop, manufacture, and sell the drug,



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

¹At present (as of Dec 31, 2024), no partner has progressed to the development and sales step.

²The 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.

³The use of ibVIS® is limited to the duration of the joint drug discovery research between our company and pharmaceutical companies.

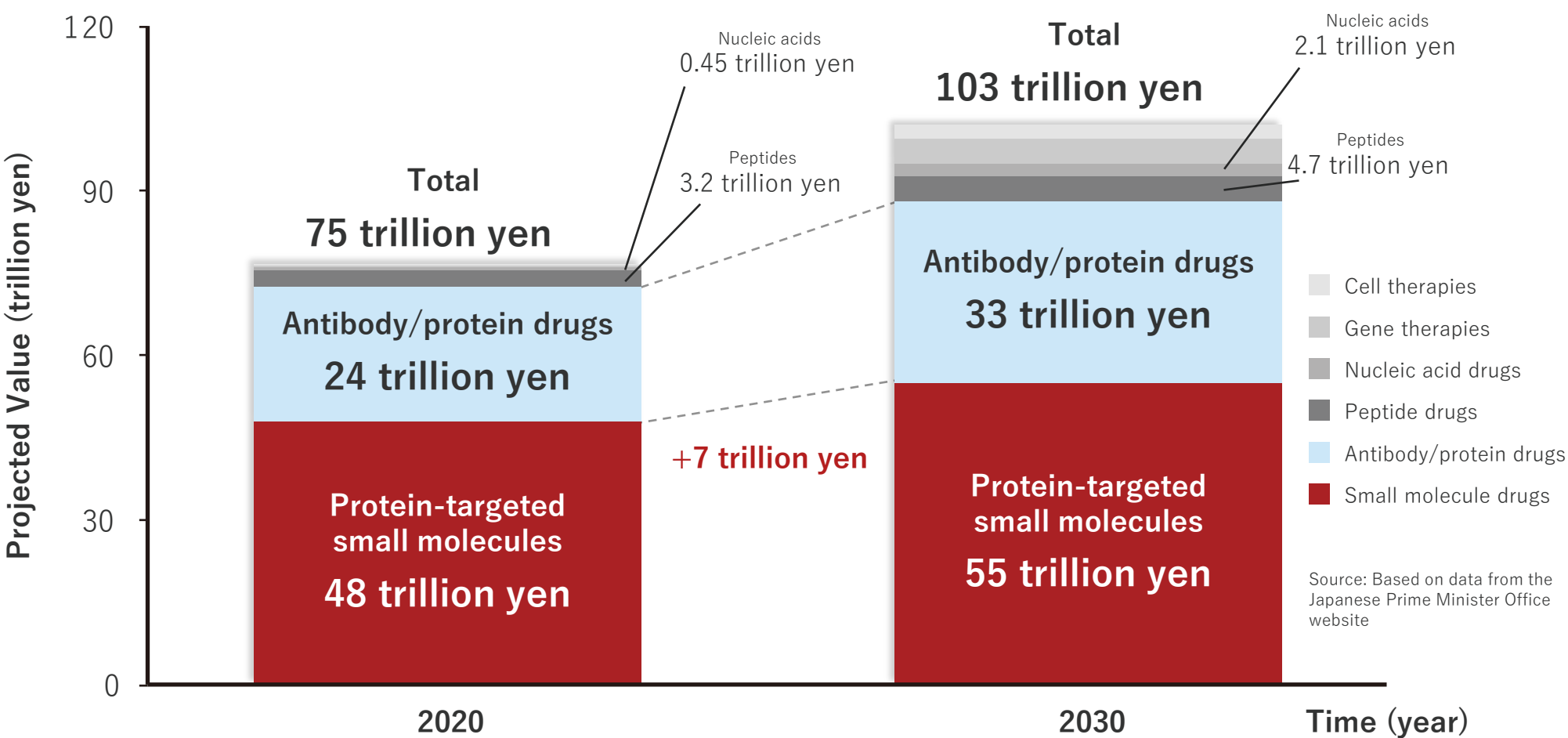
⁴In some cases, arrangements (compensations) for development, manufacturing, and sales licenses may be included at the time of the initial joint drug discovery research agreement.



External Environment

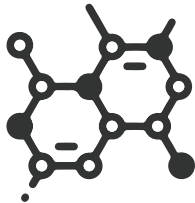
Small molecule drugs have always been at the center of the drug market

The market for small molecule drugs has reached maturity but is expected to continue growing at a steady rate. It is projected to account for more than half of the total drug market in 2030



Small molecule drugs can be administered orally and have established, low-cost manufacturing

The drug industry is a manufacturing industry. Even if a drug discovery technology is scientifically excellent, issues with mass production or cost can prevent the drug from becoming a commercial product and forming a market. Small molecule drugs, which can be administered orally and are less burdensome to patients, have an established manufacturing process and extremely low manufacturing costs

Type of drug		Medium-sized drugs/biologics			
		Small molecule drugs	Peptide drugs	Nucleic acid drugs	Antibody drugs
Form (image)					
Molecular weight		100–500	500–10,000	up to 10,000	about 100,000 and greater
Manufacturing	Method	Chemical synthesis	Chemical synthesis/ culture	Chemical synthesis/ culture	Culture
	Cost	Low	Very high	Very high	High
Drug target	Protein	☐	☐		☐
	mRNA	★		☐	
Route of administration	Oral	☐			
	Others	☐	☐	☐	☐

★ The emergence of drug discovery technologies that target mRNA, including our technology, will make implementation feasible and will serve as a catalyst for the renewed expansion of the small molecule drug market.

Growing interest in RNA-targeted small molecule drug discovery

The November 2023 issue of the magazine published by the American Chemical Society highlighted the rise of RNA-targeted small molecule drug discovery biotech companies (top right). The October 2023 issue of the same magazine featured the resurgence of small molecule drug discovery (bottom right), in which the authors opined that targeting the RNA (nucleic acid) is becoming a mainstream approach to drug discovery



DRUG DISCOVERY

The RNA drug hunters

Academics, biotech start-ups, and big pharma companies are designing small molecules that target RNA

by [Ryan Cross](#)

November 27, 2017 | A version of this story appeared in [Volume 95, Issue 47](#)

c&en
GLOBAL ENTERPRISE

DRUG DISCOVERY

Is this a golden age of small-molecule drug discovery?

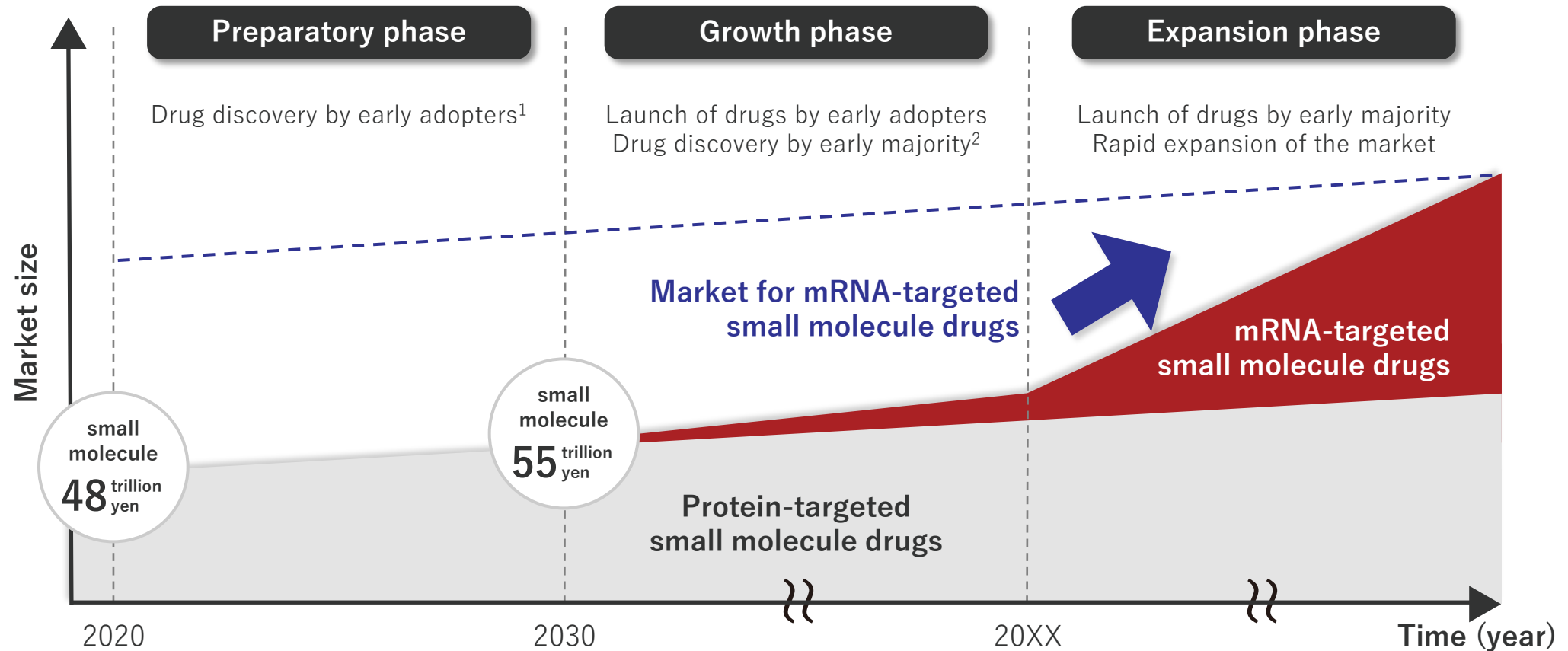
With innovative tools and techniques, it's an exciting time for medicinal chemists building new drugs

[Laura Howes](#)

C&EN, **2023**, 101 (36), pp 28–32 | October 30, 2023

Market for mRNA-targeted small molecule drugs on par with that for protein-targeted

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 and become a large drug market in the future (**expansion phase**)



¹People who buy products and services after the innovators

²People who are influenced by early adopters

Source: Estimates made by the company based on data from the Japanese Prime Minister Office website

Competitive advantage apparent in the number of partners

Platform-based companies tend to have smaller partnership sizes but have the advantage of partnering with a larger number of pharmaceutical companies. Due to the trust in our technology and the track record of partnerships, we have been able to enter larger contracts in recent years, and the total size of our partnerships is nearing that of our competitors

Company name	Business model	Partner (year partnership began)	Partnership size*	Disease area
 Veritas In Silico Established in 2016	Shift from platform to hybrid business	Toray (2021) Shionogi (2021) RaQualia Pharma (2022) Takeda (2023)	114.4 billion yen Total partnership amount	Oncology, central nervous system diseases, infectious diseases, etc.
Arrakis Therapeutics USA, Established in 2015	Pipeline	Roche (2020) Amgen (2022)	37.1 billion yen Upfront payment (n/a) Total partnership amount	Undisclosed
Ribometrix USA, Established in 2014	Pipeline	Vertex (2019) Genentech (2021)	244.3 billion yen Total partnership amount	Undisclosed
Anima Biotech USA, Established in 2014	Pipeline	Eli Lilly (2018) Takeda (2021) AbbVie (2023)	391.4 billion yen Total partnership amount	Oncology, autoimmune diseases, central nervous system diseases

As of Dec. 31, 2024

*Converted at 1USD= 140 yen

Source: Based on information from Crunchbase and the websites of the competitor companies.

Harnessing AI to lead the world in RNA-targeted small molecule drug discovery

Our company is regarded overseas as a leader in maximizing the potential of AI-based RNA-targeted small molecule drug discovery

Our CSO Morishita has published a review article on the use of AI in response to a request from an international journal

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



REVIEW

OPEN ACCESS Check for updates

Recent applications of artificial intelligence in RNA-targeted small molecule drug discovery

Ella Czarina Morishita and Shingo Nakamura

Veritas In Silico Inc, Tokyo, Japan

ABSTRACT

Introduction: Targeting RNAs with small molecules offers an alternative to the conventional protein-targeted drug discovery and can potentially address unmet and emerging medical needs. The recent rise of interest in the strategy has already resulted in large amounts of data on disease associated RNAs, as well as on small molecules that bind to such RNAs. Artificial intelligence (AI) approaches, including machine learning and deep learning, present an opportunity to speed up the discovery of RNA-targeted small molecules by improving decision-making efficiency and quality.

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.

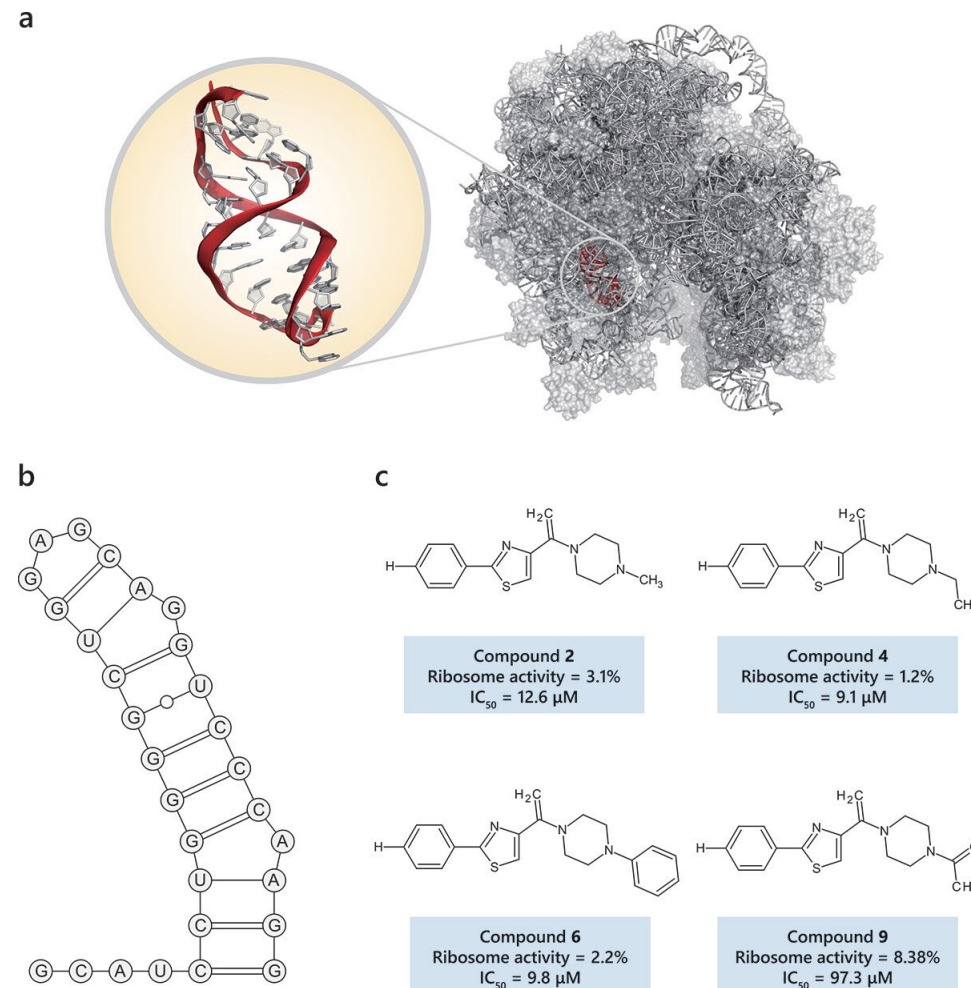
Expert opinion: Key areas for improvement include developing AI tools for understanding RNA dynamics and RNA – small molecule interactions. High-quality and comprehensive data still need to be generated especially on the biological activity of small molecules that target RNAs.

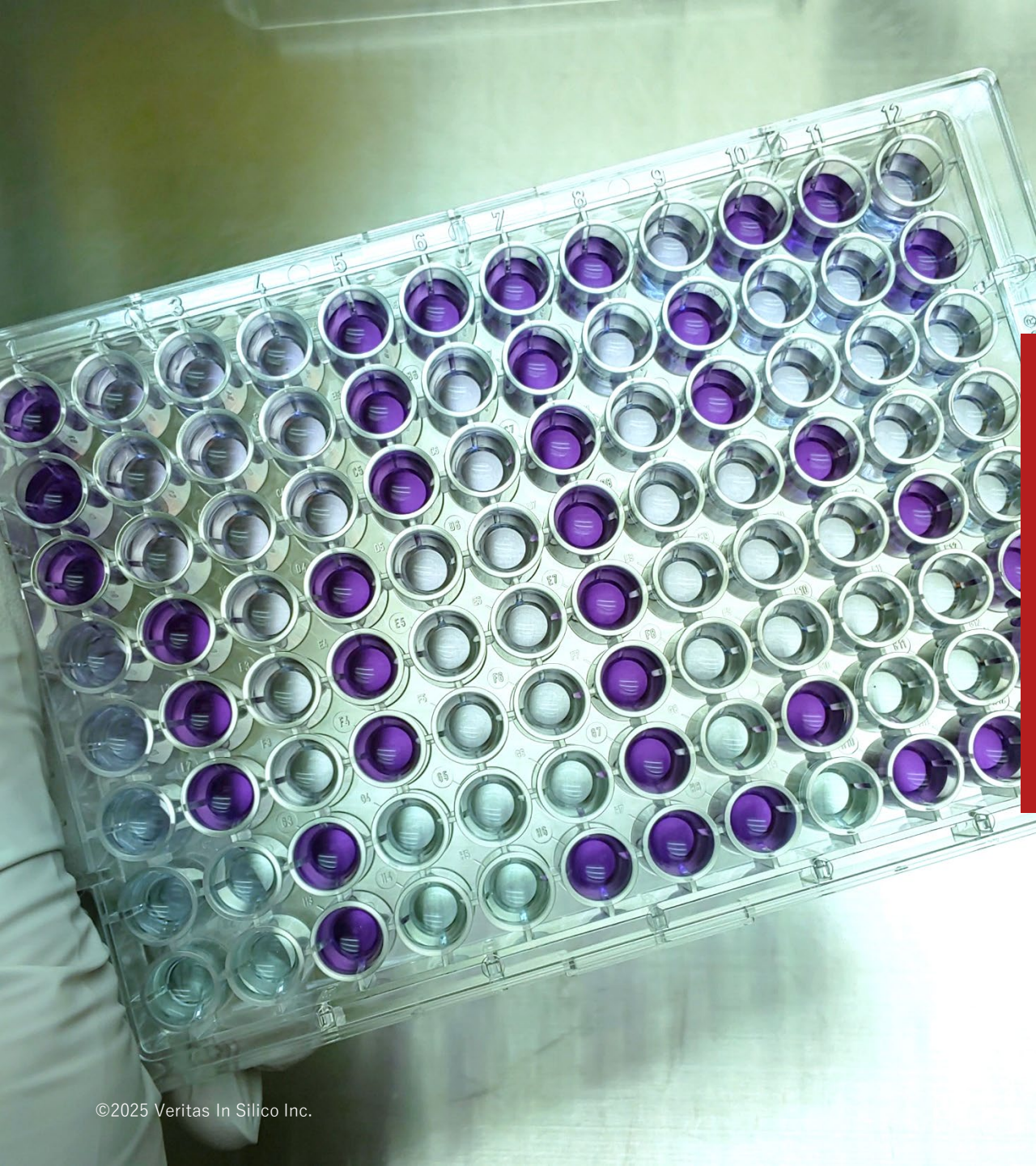
ARTICLE HISTORY

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



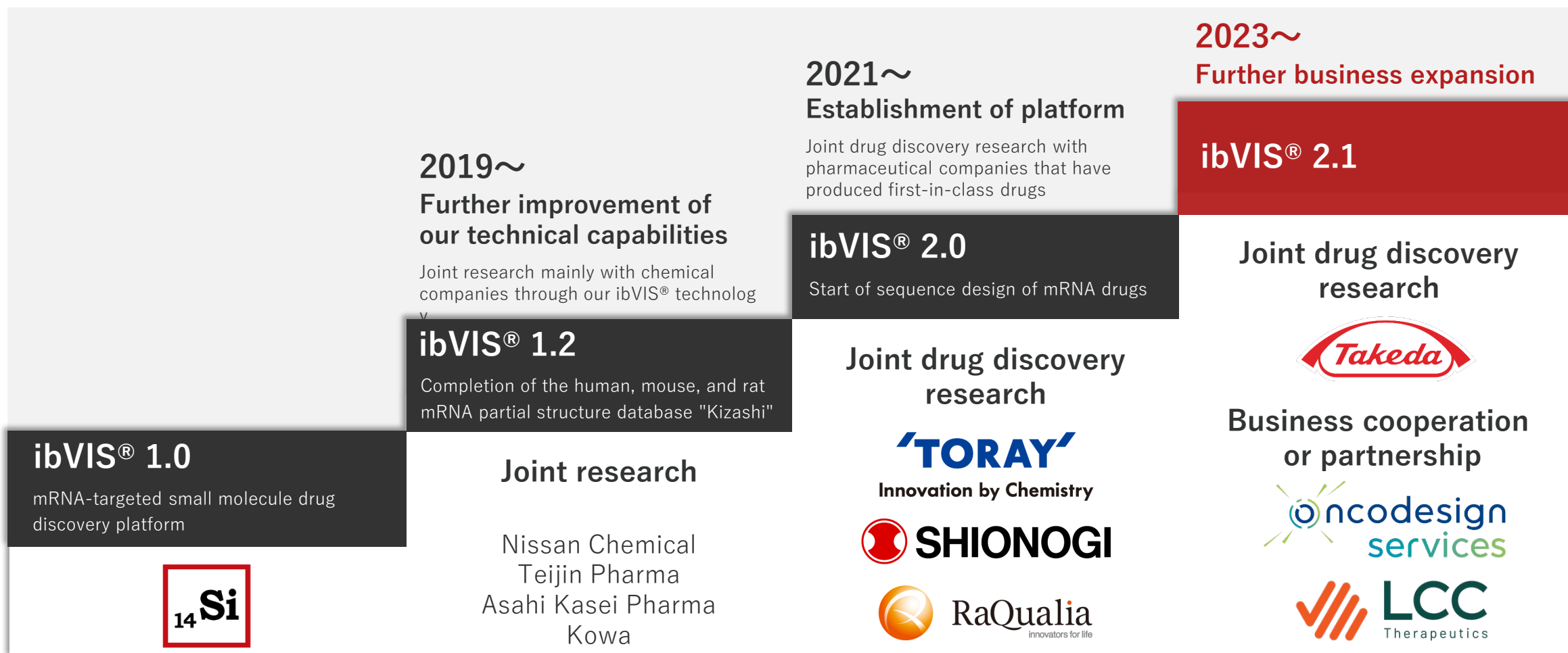


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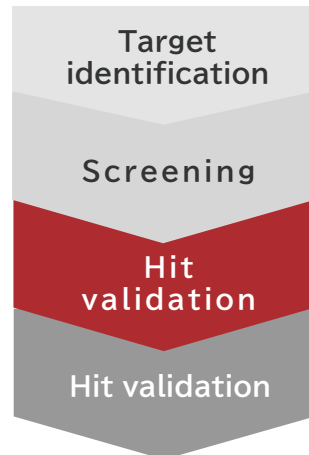
Business Progress

Further business expansion through joint research/drug discovery research with pharmas

We successfully improved the technical capabilities of ibVIS® by leveraging the characteristics of our platform business (from 2019), enabling us to enter into more profitable joint drug discovery research agreements (from 2021). Through our business cooperation with Oncodesign Services and partnership with LCC Therapeutics, we aim to further expand our business (from 2023)



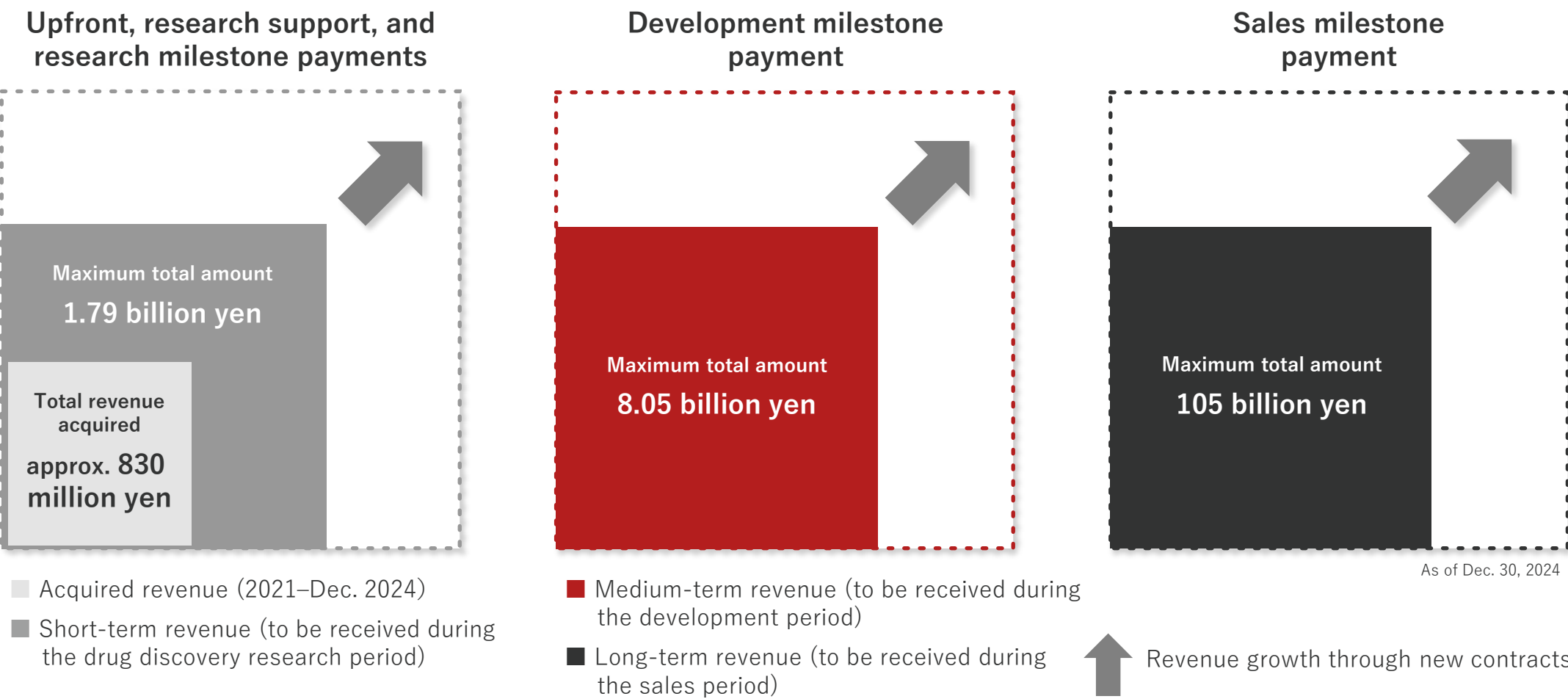
Proven track record in joint drug discovery projects with pharmaceutical companies

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		• Contract amount undisclosed • VIS obtains rights to the compounds*
	November 2021	Infectious diseases, psychiatric and neurological disorders		• Up to 85 billion yen contract • Royalties
	December 2022	Cancer		• Contract amount undisclosed • Royalties
	June 2023	Undisclosed		• Contract amount undisclosed • Royalties
			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 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

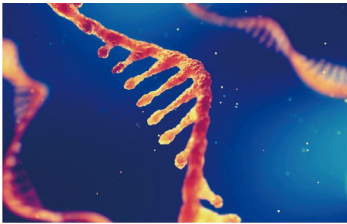


Business progress in FY2024

Mar.

Efforts to Create In-house Pipeline of Nucleic Acid Drugs

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



Jun.

Milestone Achieved

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



Jun.

Steady Progress in Compound Discovery

Obtained several small molecule compounds with target profiles through cell-based assays in joint drug discovery research with RaQualia to create small molecule drugs for cancers



Sep.

Milestone Achieved

Achieved a milestone in joint drug discovery research with Shionogi to create mRNA-targeted small molecule drugs for infectious diseases and psychiatric and neurological disorders



Dec.

Collaboration Development & Commercialisation Agreement

Partnered with LCC Therapeutics to create shared pipeline of mRNA-targeted small molecule drugs for cancers and CNS disorders for development and future commercialisation of these drugs



Collaboration Development & Commercialisation Agreement with LCC

LCC Therapeutics (renamed from Liverpool ChiroChem Ltd. in January 2025) possesses an innovative chemical platform for the rapid synthesis of small molecule compounds. Partnering with LCC, which excels in chemical platforms, enables us to efficiently create pipeline of small molecule drugs

URL: <https://contents.xj-storage.jp/xcontents/AS82025/326f6e81/928c/4803/8278/580e6f5e1a64/140120241220542051.pdf>



Aim of the agreement between VIS and LCC

The partnership utilizes LCC's advanced chemical platform, PACE™, and our mRNA-targeted small molecule drug discovery platform, ibVIS®

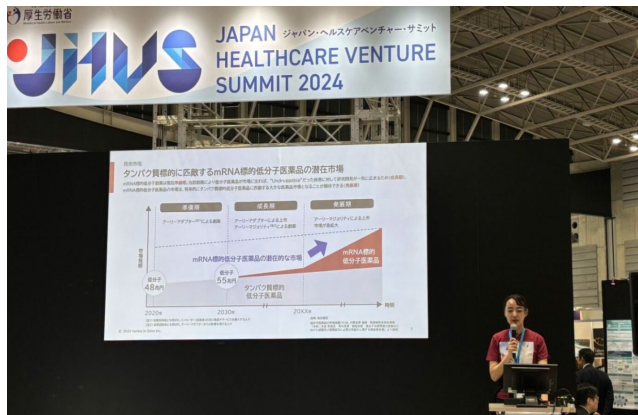
We aim to create blockbuster drugs targeting large markets, such as the oncology field, by leveraging the technological capabilities of both companies

We aim to efficiently create pipeline of mRNA-targeted Small molecule drugs and share the rights to the compounds

Participation in lectures and exhibitions in FY2024

We have undertaken various initiatives to acquire new partnerships

Japan Healthcare Venture Summit (JHVS) 2024



We exhibited our company booth at JHVS 2024, held by the MHLW at Pacifico Yokohama from October 9 to 11, 2024, as well as introduced our mRNA-targeted small molecule drug discovery business in the JHVS SHOWCASE on the first day of the event.

BIO-Europe 2024 Company Presentations Session



Our CEO Nakamura and CSO Morishita joined BIO-Europe 2024, held in Stockholm, Sweden, from November 4 to 6, 2024. Not only did they introduce our platform technology to pharma from the EU and US, but CSO Morishita delivered a presentation at the Company Presentations Session.

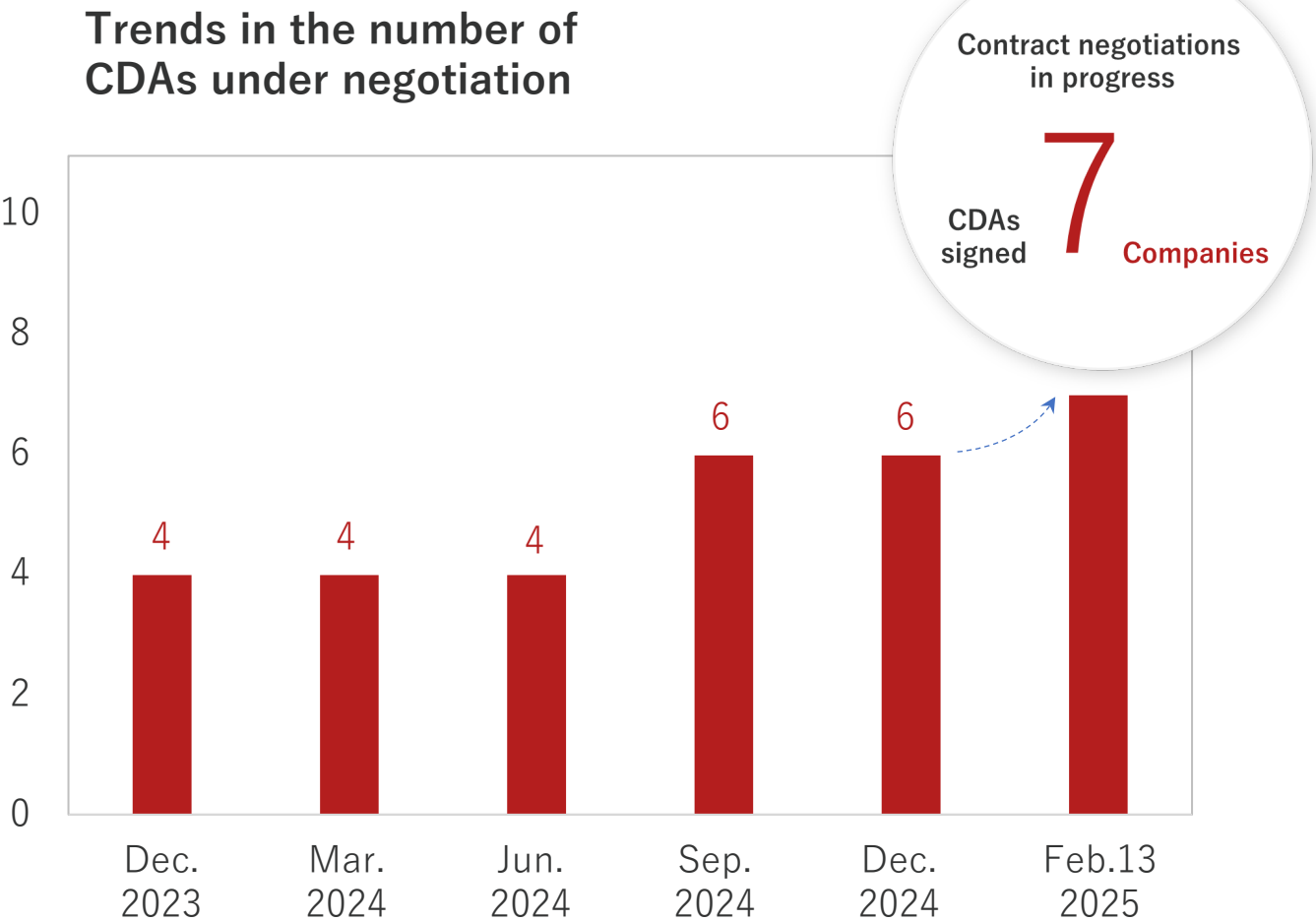
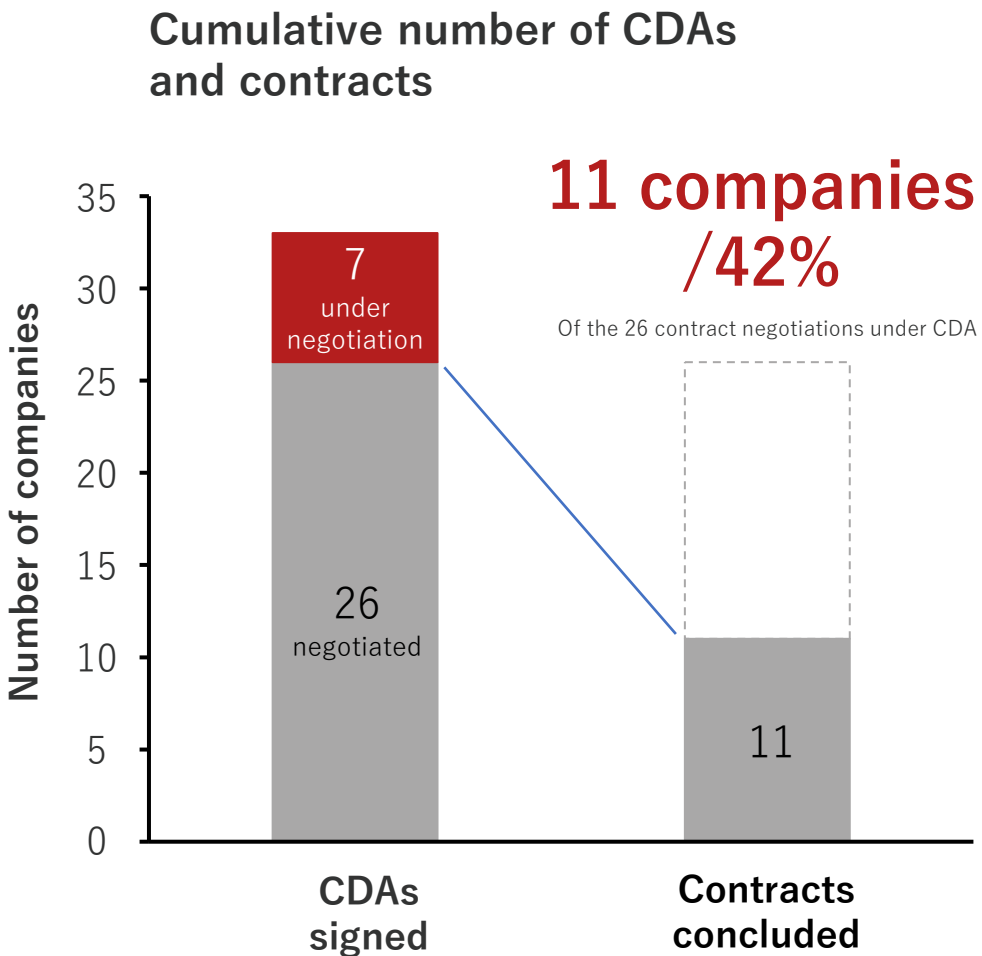
The 41st Medicinal Chemistry Symposium

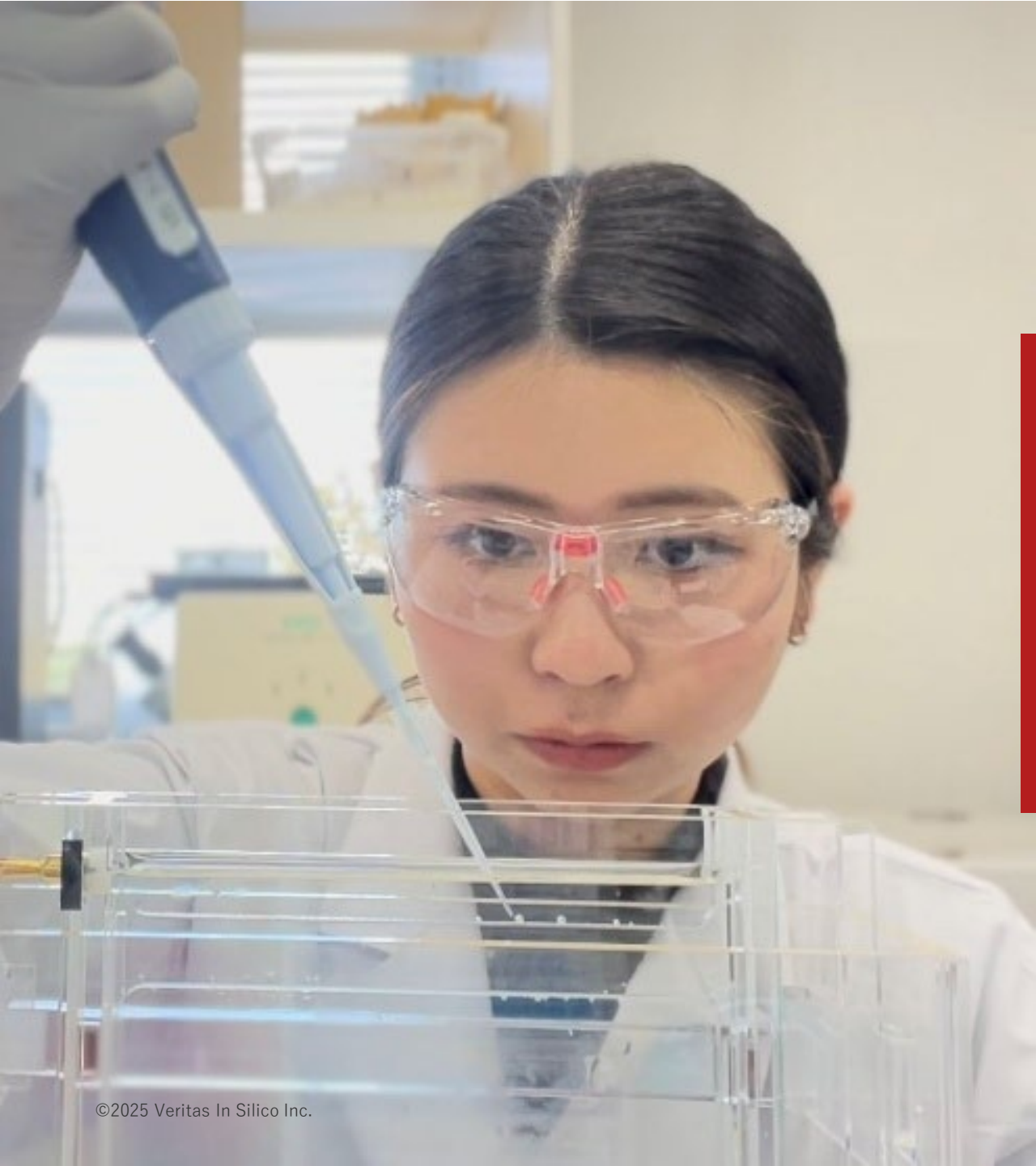


We participated in the 41st Medicinal Chemistry Symposium at Kyoto TERSA from November 20 to 22. Together with the display of our company booth, Our CEO Nakamura gave an invited lecture, and our Principal Investigator Otsu explained our platform technology at the poster session.

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 conclusion as a result of negotiations under the CDAs is approx. 42%, and the median time between signing a CDA and reaching a contract conclusion is 14 months
Our business development policy is to ensure a commensurate number of CDAs enough to conclude contracts with 2 companies each year after 2025

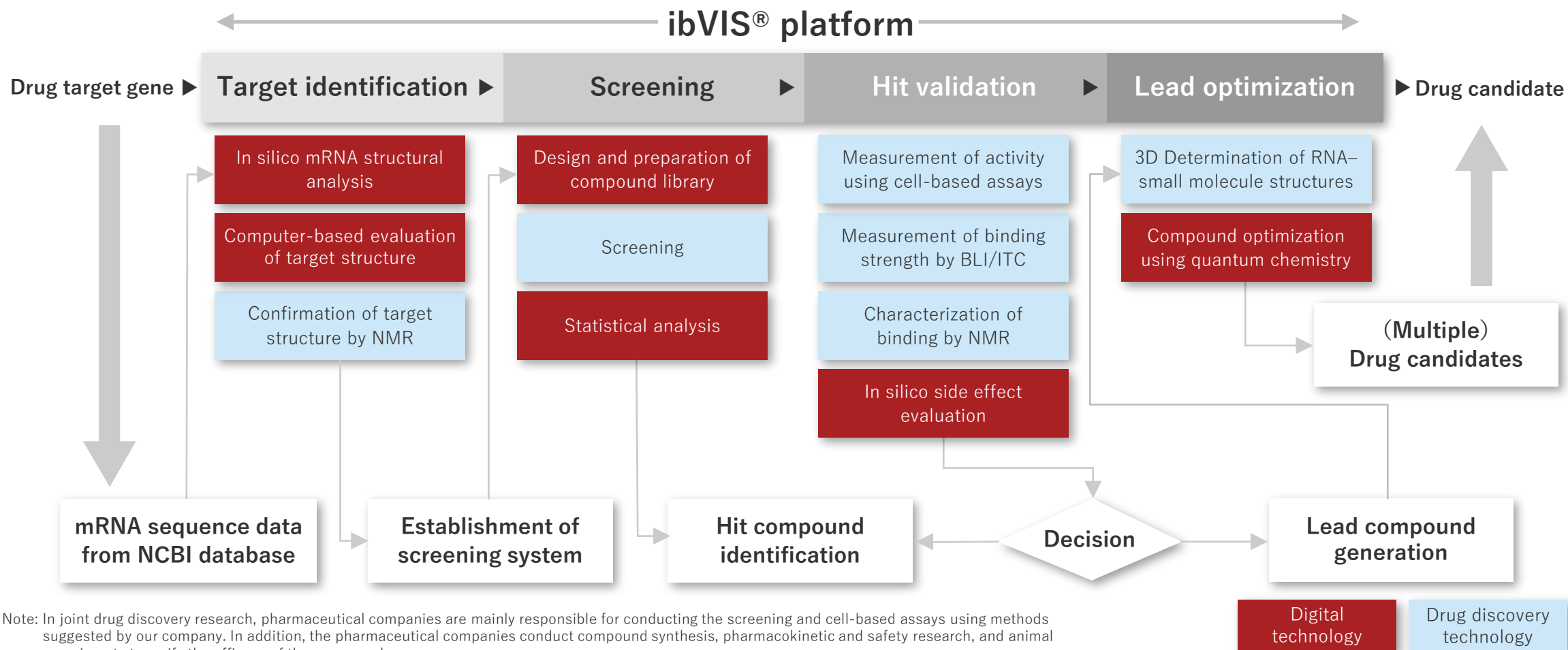




5 Drug Discovery Platform

One-stop platform based on verified and proven technologies

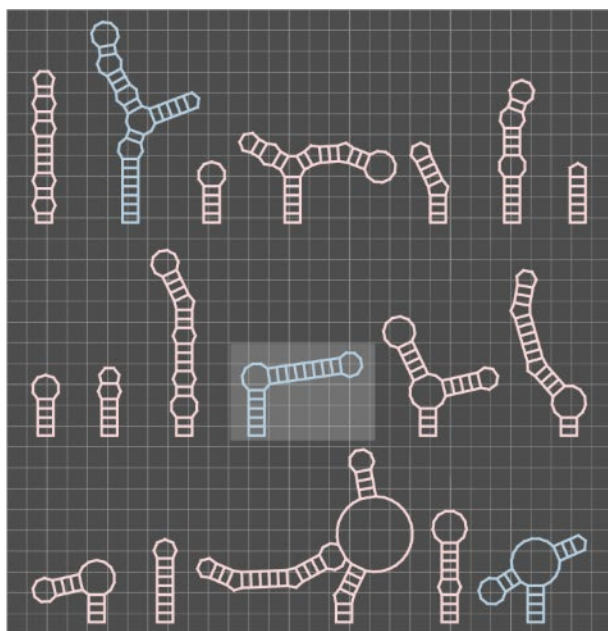
ibVIS® consists of a series of drug discovery and digital technologies, enabling the one-stop implementation from mRNA sequence data to the acquisition of drug candidate compounds. We already have a proven track record of up to hit validation in joint drug discovery research, etc. and have proven the utility of our lead optimization technologies through in-house research



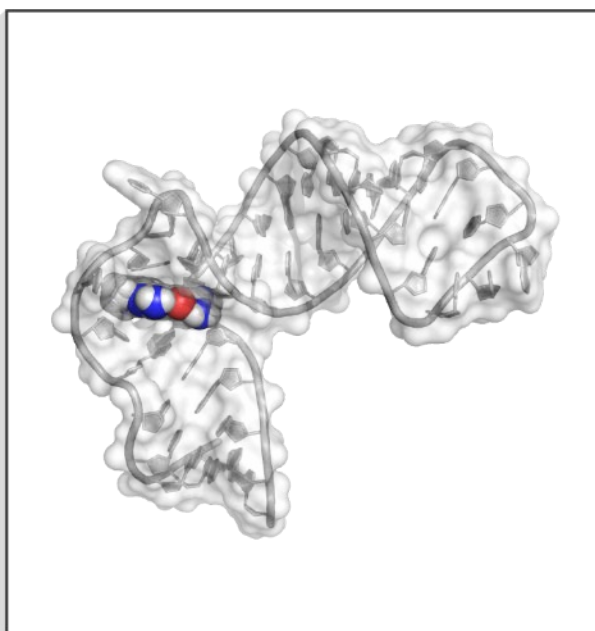
ibVIS® target identification to maximize the potential of mRNA targets

Target identification with ibVIS® is based on our proprietary in silico (using computers) RNA structural analysis, which enables us to rapidly and accurately identify multiple drug targets (target structures) from mRNAs selected by the pharmaceutical companies. It is one of our competitive advantages

In silico mRNA structural analysis



Target for small molecules mRNA target structure



01 Fast

Target structures on mRNA can be identified in significantly less time than using experimental methods

02 Accurate

The target structures identified using our in silico technologies have an accuracy of >90%, while other computational methods have an accuracy of only 60–70% based on our evaluation

03 Well-validated targets

The target structures that we select are rigorously tested to ensure they are optimal targets for small molecule drug discovery

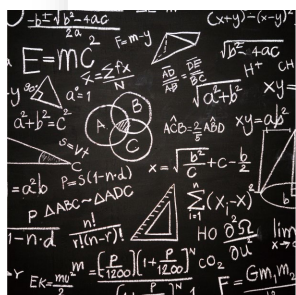
Elusive nature of mRNA revealed by statistical mechanics and thermodynamics theories

Conventional drug discovery focuses on biology and chemistry. Twenty years of experience and achievements of our representative Nakamura and research and validation for practical application by our researchers led to introducing more microscopic theories of physics—statistical mechanics and thermodynamics—into the existing drug discovery research, making it possible to analyze the structure of the mRNA and to search for drug targets

Theory/Computation ← → Actual/Experimentation

VIS drug discovery

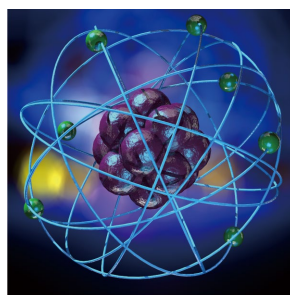
Mathematics <
Quantum mechanics



Theory

Roughly 0.1 nm

Physics



Atom

Statistical
mechanics

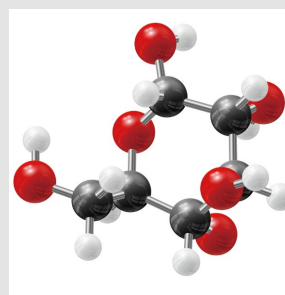
Thermodynamics

Conventional drug discovery

several nm–several 10 nm

80 nm–0.2 mm

Chemistry



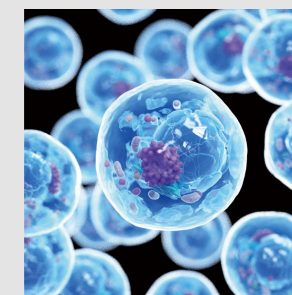
Molecule

Molecular biology



mRNA/Protein

Biology



Cell

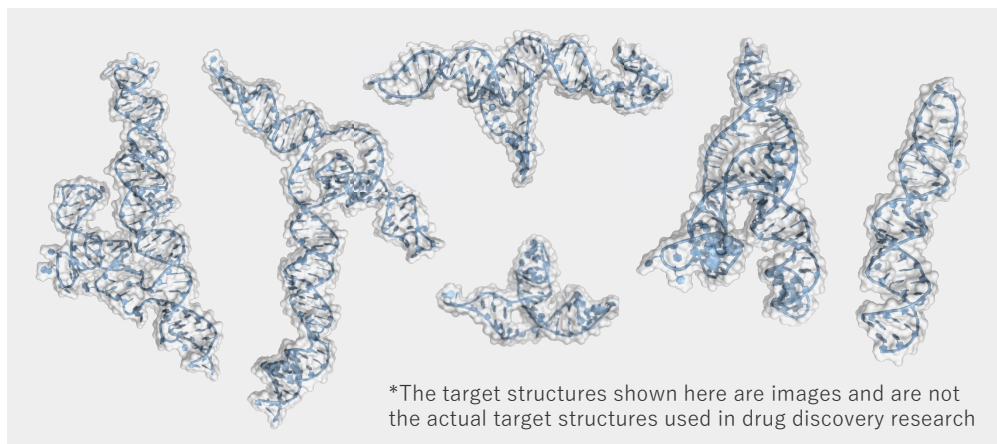
Note: nm = 10⁻⁶ mm

ibVIS® screening technology proven to have a high probability of success

In drug discovery projects with ten pharmaceutical companies, we screened tens to hundreds of thousands of compounds for cancer, CNS diseases, infectious diseases, etc., and conducted about fifty screening projects in five years. We found target structures on the mRNAs (left), conducted screening against them (right), and as a result obtained hit compounds with a success rate of about 95%

As of early Dec. 31, 2024

Target identification

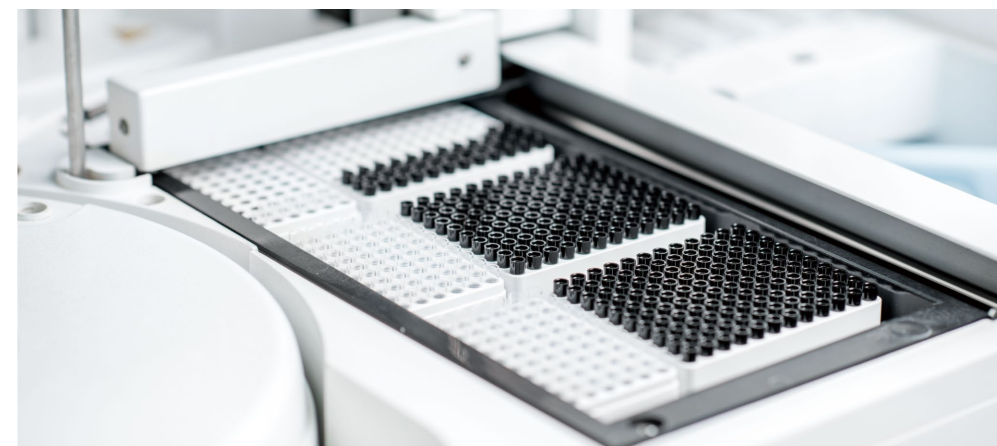


Identify multiple mRNA target structures for small molecules

Success rate **98%**



Screening



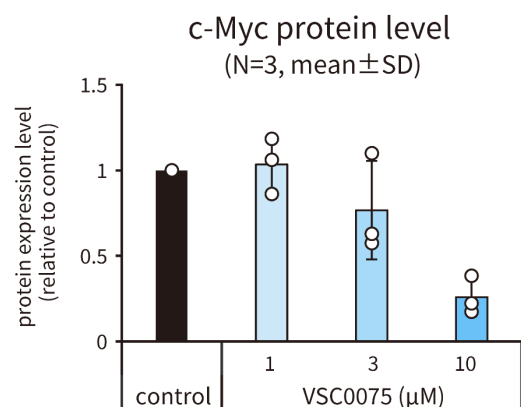
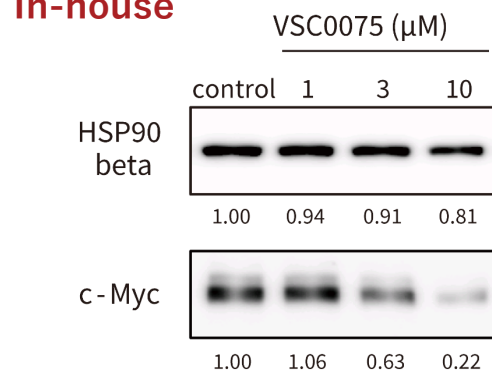
Perform fast and sensitive screening (qFRET) against mRNA target structures and obtain multiple hit compounds showing activity

Success rate of Screening **96%**
(When >20,000 compounds are used 100%)

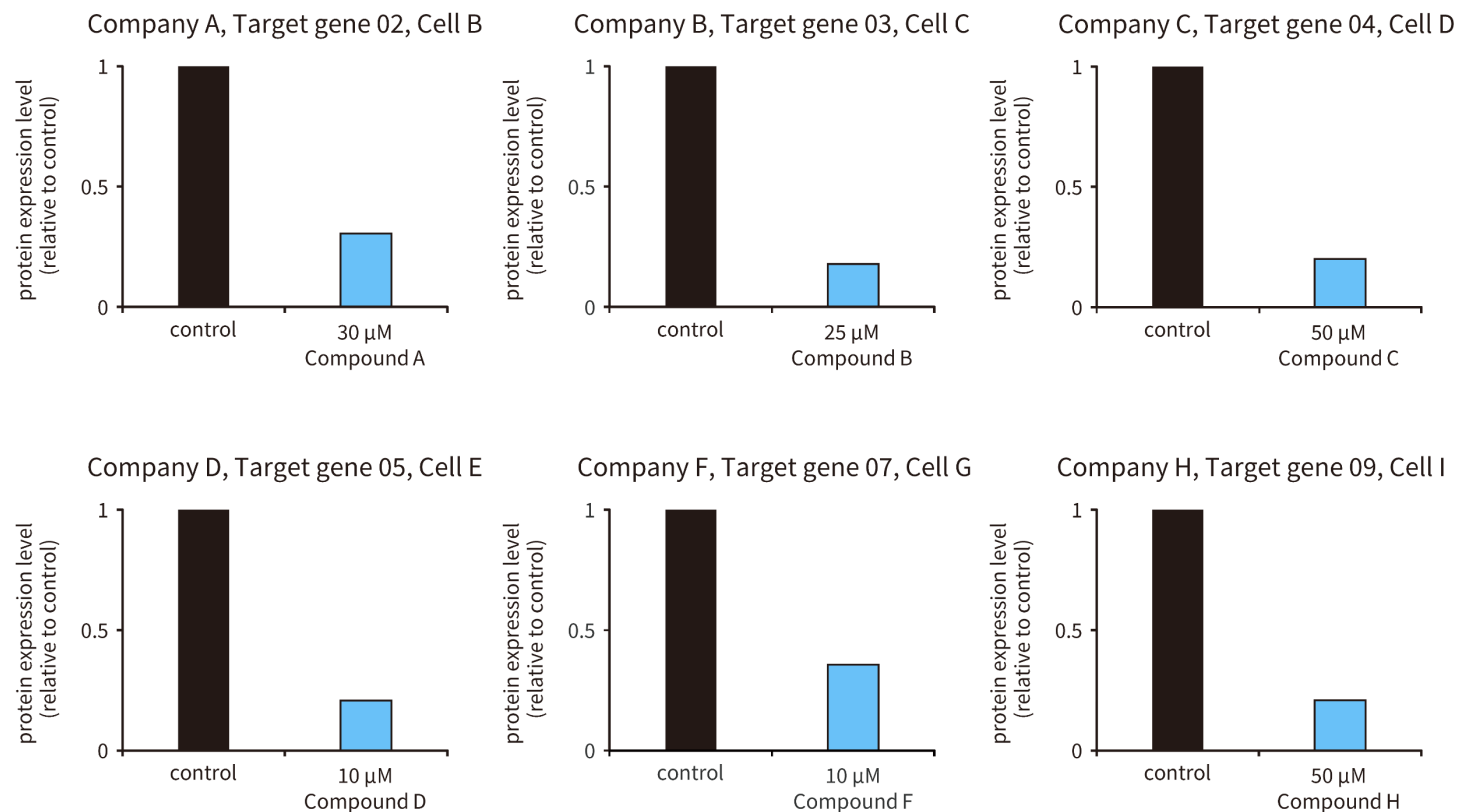
Hit compounds obtained from screening exhibit cellular activity

Hit compounds obtained from in-house research and projects with 10 pharmaceutical companies show cellular activity, a unique consideration for mRNA-targeted small molecule drug discovery. The effects of the hit compounds are then verified using various RNA measurement methods (p. 37).

In-house



With pharmaceutical companies



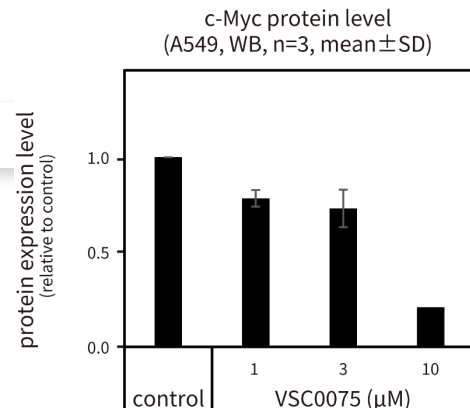
Left: Addition of the compound (VSC0075)—obtained in the screening against a target structure found on the mRNA of a disease-related protein (c-Myc)—to the cells resulted in a predominant decrease of the c-Myc protein. HSP90 beta, a representative of common proteins, was hardly affected. Right: Cell-based assay results of the compounds (Compound A–H) obtained in the screening for six partners are shown, with one example for each.

Various measurement techniques to examine the binding of mRNA and compounds in detail

To obtain compounds that show higher activity and desirable characteristics as drugs, we use various RNA measurement techniques to analyze the mode, strength, and characteristics of binding between the hit compounds (those that show cellular activity) or newly synthesized compounds and the mRNA

Cell-based assay

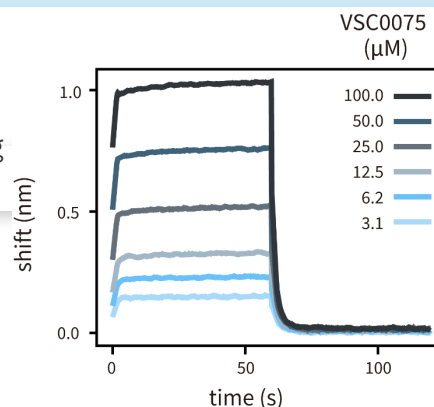
Determine activity in cells



Note: Images shown here were created from internal data concerning the compound VSC0075.

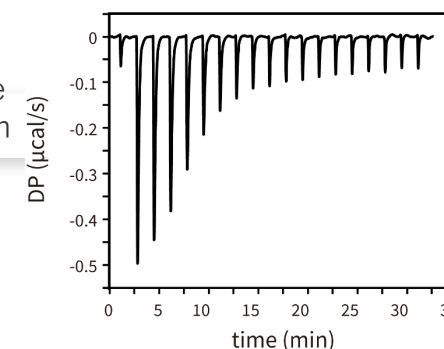
BLI measurement

Examine the binding rate between the RNA and the compounds



ITC measurement

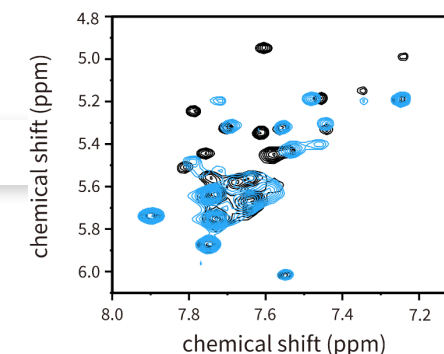
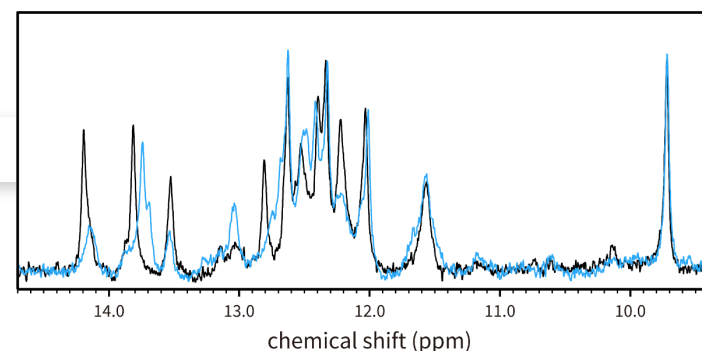
Precisely determine the binding strength of the compounds to the RNA



NMR measurement

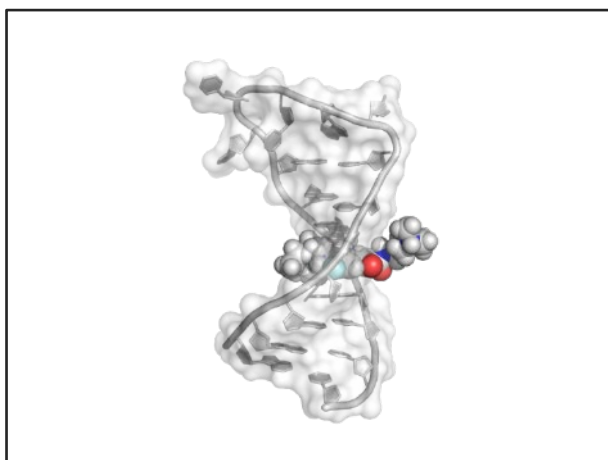
Investigate the binding sites of the compounds on the RNA

--- With small molecule
--- Without small molecule



Lead optimization using RNA structure determination and state-of-the-art computations

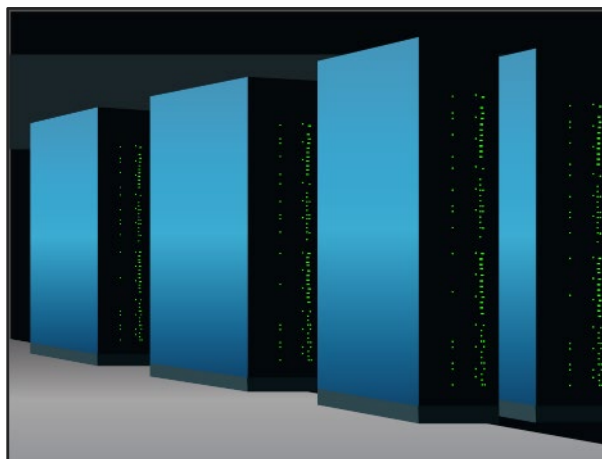
RNA-tailored structure determination technology



We are uniquely equipped to perform RNA 3D structure determination, with our scientists having rare expertise in RNA X-ray crystallography and NMR spectroscopy



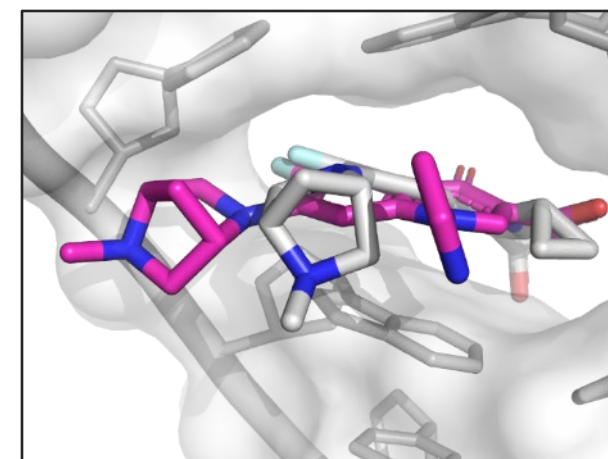
RNA –small molecule interaction analysis technology



We use Japan-made state-of-the-art methods and the Fugaku supercomputer to thoroughly analyze the interactions between mRNA targets and small molecule binders



Rational optimization of mRNA-targeted small molecules

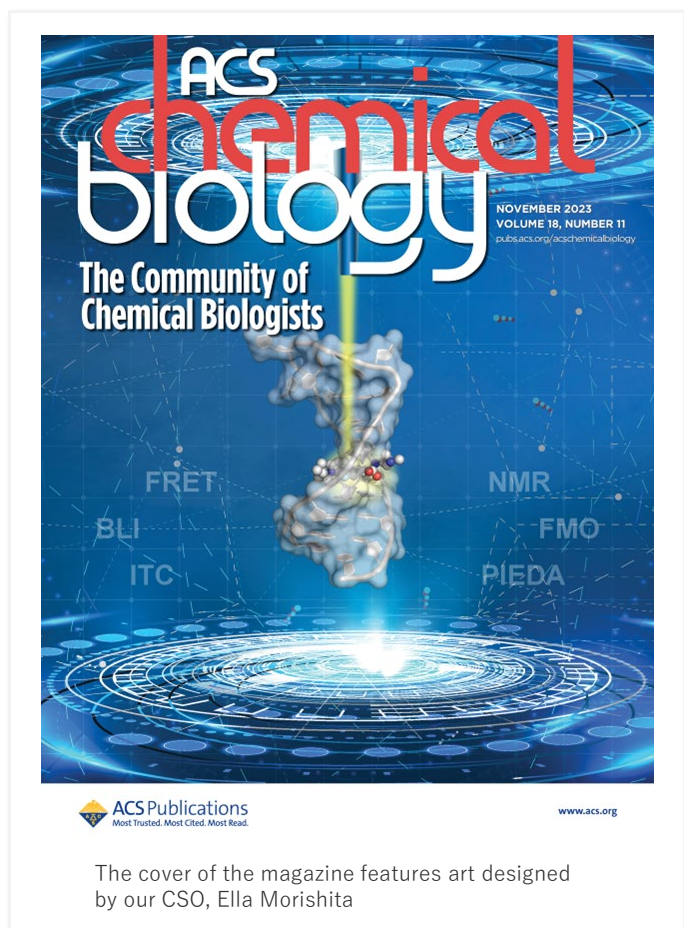


Our technologies enable us to rationally design small molecules with higher potency and selectivity for mRNA. As a result, we can optimize small molecules faster and more efficiently than traditional methods

Novel compounds that bind stronger to RNA structures designed using digital technology

Our joint publication with Prof. Fukuzawa of Osaka University was published in the November 2023 issue of ACS Chemical Biology. This is the first example showing the correlation between the binding of small molecule compounds to RNA derived from experiments and fragment molecular orbital (FMO*) calculations. The paper demonstrates the usefulness of FMO calculations for the rational design of mRNA-targeted small molecule drugs

*FMO is a type of quantum chemical calculation method



pubs.acs.org/acschemicalbiology

Articles

Probing RNA–Small Molecule Interactions Using Biophysical and Computational Approaches

Amiu Shino, Maina Otsu, Koji Imai, Kaori Fukuzawa, and Ella Czarina Morishita*

Cite This: *ACS Chem. Biol.* 2023, 18, 2368–2376
 Read Online

Note: VIS scientists

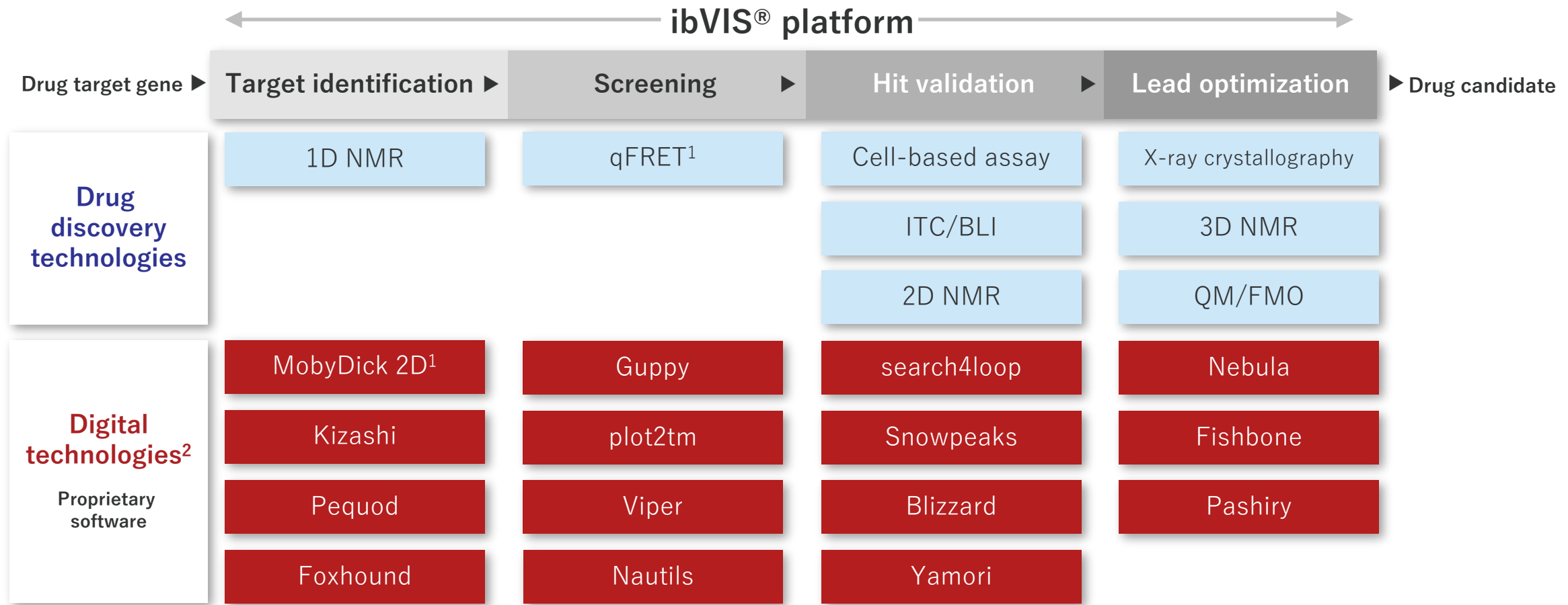
Parts of the results published in the paper

The parent compound obtained from screening

Newly designed compounds predicted to have higher activity

Patents and digital technologies provide double assurance for ibVIS® exclusivity

Our ibVIS® platform is composed of drug discovery technologies modified for mRNA and various digital technologies developed as software based on the know-how of our scientists. The exclusivity of ibVIS® is doubly secured by patents covering some of the drug discovery and digital technologies and by the confidentiality of the proprietary digital technologies



¹Patent 6781890, "Method for screening compound for controlling RNA function"

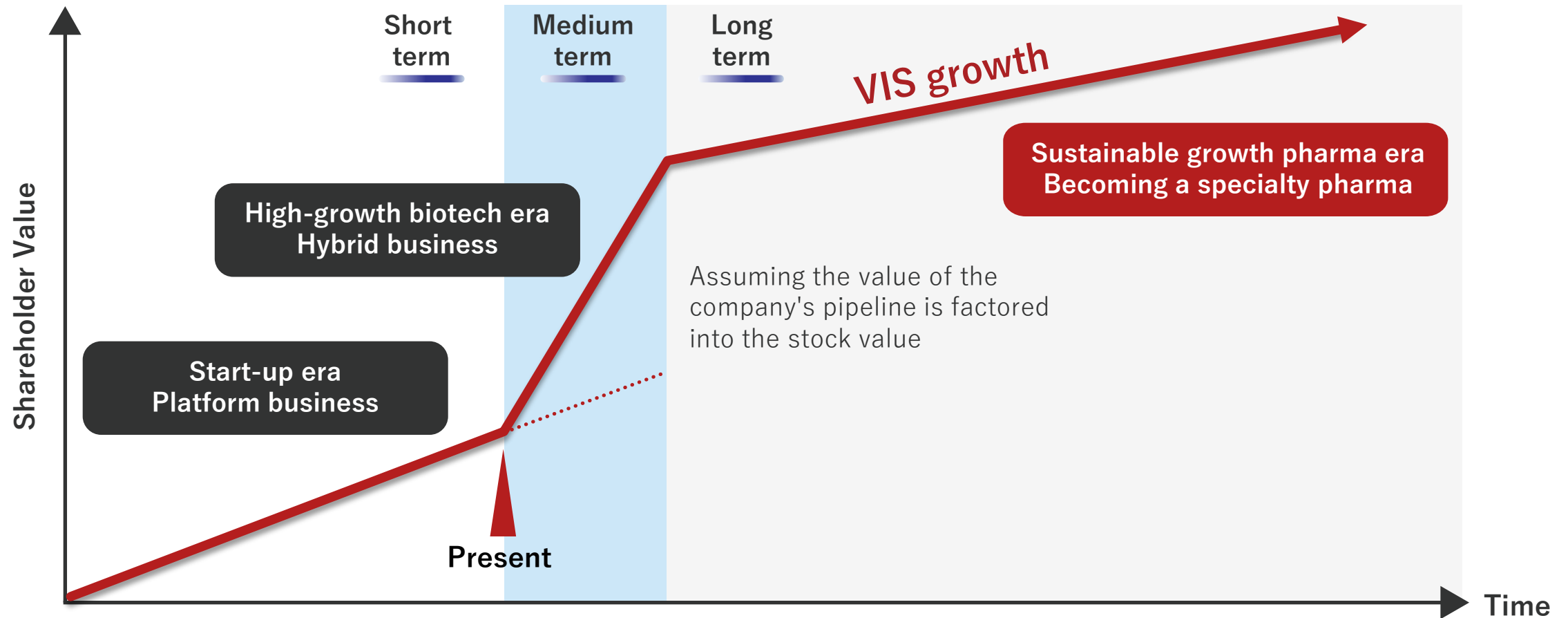
²Proprietary software to support large-volume data analysis and experiments



6 Growth Strategy

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

We are transitioning from our current platform business to a hybrid business model aimed at achieving high growth rates. For sustainable growth, we plan to evolve into a specialty pharmaceutical company with capabilities in research, development, and sales of drugs, focusing on mRNA-related drug discovery.



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

Sustainable growth through diversification of mRNA-related drug discovery business

Our in silico RNA structural analysis can be applied to various mRNA-related drug discovery approaches. When we transition to the hybrid 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** →

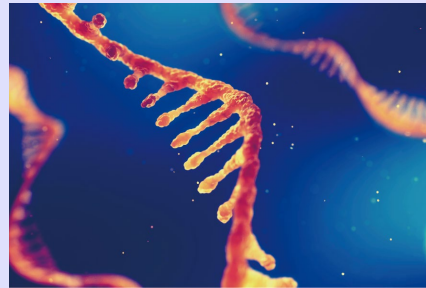
01



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, a solution to the need for an alternative to protein replacement therapy

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

Annual goals during the mid-term management plan towards VIS' 2030 Vision

Our vision for 2030 is to establish ourselves as a specialty pharmaceutical company. We will implement the following measures during our medium-term management plan from 2025 to 2027 to achieve this vision.

Conventional KPIs

- Amount of business revenue²
- Target number of new contracts

Annual target **2 contracts** → FY2024 results **1 contracts**

FY2024

Progression of
platform business

Preparation for
hybrid business

FY2025

Hybrid business

- Acquire 4 new contracts⁴
- Create the 1st pipeline
- Prepare for relocation of Shin-Kawasaki Research Institute

FY2026

- Acquire 2 new contracts
- Create the 2nd pipeline
- Commence preclinical test
- Complete relocation of Shin-Kawasaki Research Institute

FY2027

- Acquire 2 new contracts
- Create the 3rd pipeline
- Continue preclinical test

FY2030

**Establishing a position
as a specialty pharma**

KPIs for the medium-term management period

- Target number of new contracts per year¹
2 contracts
- Target number of pipeline per year²
1 pipeline
- Amount of business revenue³

¹ This is a benchmark for measuring the progress of the platform business

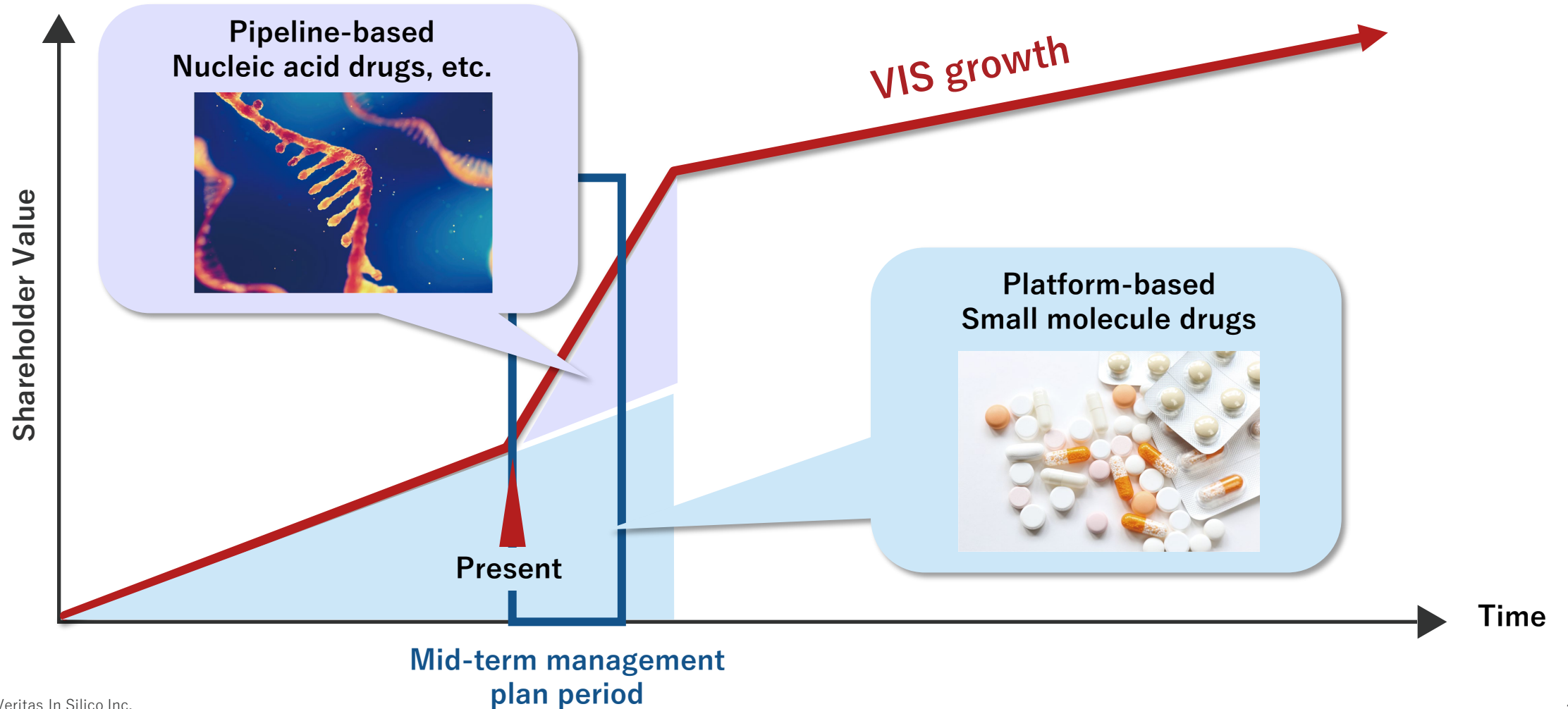
² This was set as a new KPI for the start of the pipeline business in FY2025
The creation of a pipeline is counted as the application of a patent

³ This is a benchmark for measuring the balance between progress in the platform business and the pipeline business

⁴ We aim to acquire a total of 4 contracts in FY2025, conclusion of 2 new contracts as well as the 2 contracts that had been scheduled to be concluded in FY2024. One of the 2 new contracts was signed with LCC ahead of schedule in December 2024.

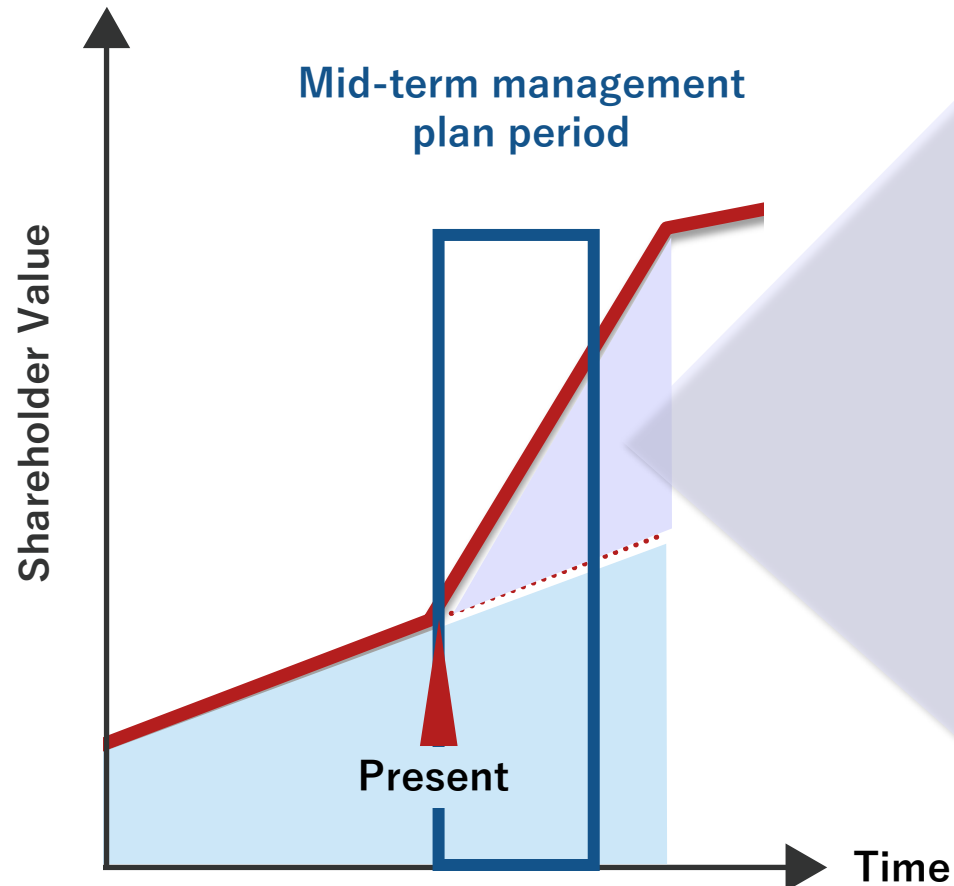
Transition to hybrid business as a prelude to specialty pharma

We aim to increase our shareholder value by expanding our platform business for mRNA-targeted small molecule drugs while creating our own pipeline with nucleic acid drugs as the main candidates



Initiatives to commence and streamline the creation of high-value pipelines

We will start projects with high current value by making use of the technical strengths of our ibVIS® platform, which allows us to target almost any mRNA



Policy for creating a pipeline

Creating pipeline that contributes to our current stock value

- Those with a large total future value
- Those with a short time to market
- Measures to reduce the most recent costs that have a large impact on current stock value

First pipeline in FY2025

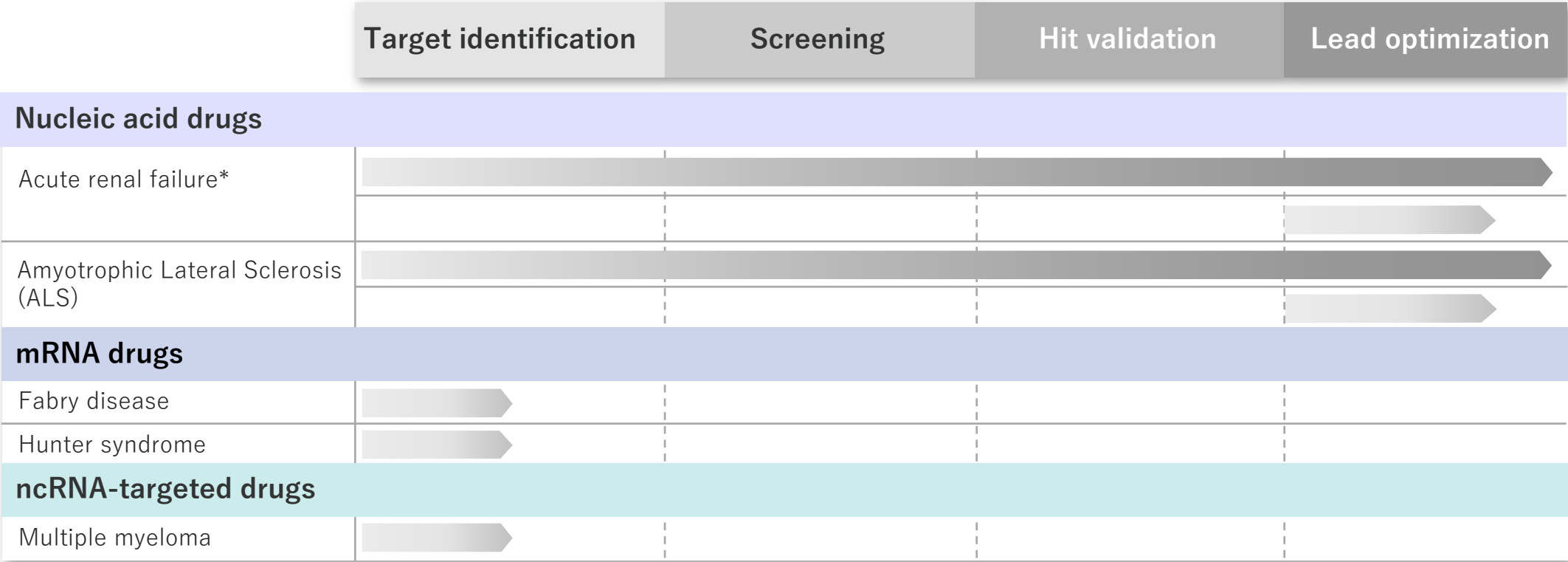
- Modality: nucleic acid drugs with short research periods
- Disease area: rare diseases to shorten development period until market launch

More efficient creation of pipeline

- To share research work and costs with partner companies
- To utilize joint research with academia, etc.

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

We have built technological capabilities to create nucleic acid drugs in as early as 8 months and already have two nucleic acid drug projects as candidates for our in-house pipeline scheduled for 2025, mainly for rare diseases



*WO2021/002359 Nucleic acid drug and use thereof

MOU with Mitsubishi Gas Chemical on research, development and manufacturing of nucleic acid drugs

Joint business with Mitsubishi Gas Chemical (MGC) will contribute to the improvement of nucleic acid drug quality and the commercial manufacturing process management. It is expected that the development phase will progress smoothly and the probability of success will increase

URL: <https://contents.xj-storage.jp/xcontents/AS82025/cb870688/daf3/4ee3/8a89/e0f000d054e0/140120241002592963.pdf>



Aims of VIS

To create pipelines of nucleic acid drugs as quickly as possible, ultimately obtaining substance patents

Aims of MGC

To establish manufacturing methods for nucleic acid drugs that contribute to new CDMO business

Contents of MOU with MGC announced Oct. 9

MGC is considering entering the nucleic acid CDMO business, seeing nucleic acid drugs as a growth market

By considering the manufacturing and purification during discovering nucleic acid drugs, we aim to ensure quality and reduce costs of nucleic acid drugs without established efficient synthesis methods

Both companies continue to discuss the process of launching the joint business

Overseas expansion by leveraging our platform business track record in Japan

Following the agreement with Takeda Pharmaceutical Company in June 2023, we will further expand our business by acquiring overseas pharmaceutical companies as drug discovery partners through business cooperation with Oncodesign Services, a French CRO, and partnership with LCC Therapeutics in the U.K.



As of February 13, 2025

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 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 science and technology and fostering a corporate culture that is rewarding to work in

Initiatives related to our business activities

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

- ◆ 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

- ◆ 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 have built a good relationship with academia, have improved our drug discovery technology and, by extension, contribute to the improvement of science and technology in Japan. In addition, we actively engage in realizing a sustainable society by making proposals for the development of biotechnology companies at the Science Council of Japan

Collaboration with academia on mRNA

Osaka University 2 projects

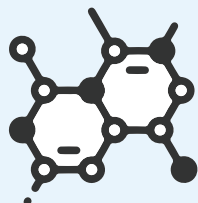
Chiba Institute of
Technology

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

Institute of Science Tokyo

Chiba Institute of Technology

CEO presentations conducted in 2024

The Science Council of Japan

JASIS 2024 Seminar hosted by MEXT

Nikkei Business Forum

Japan Bioindustry Association (JBA)

The Society of Synthetic Organic
Chemistry, Japan

mRNA Targeted Drug Discovery
Research Organization

Frontiers of Drug Discovery by LUNK-J

The 41st Medicinal chemistry Symposium

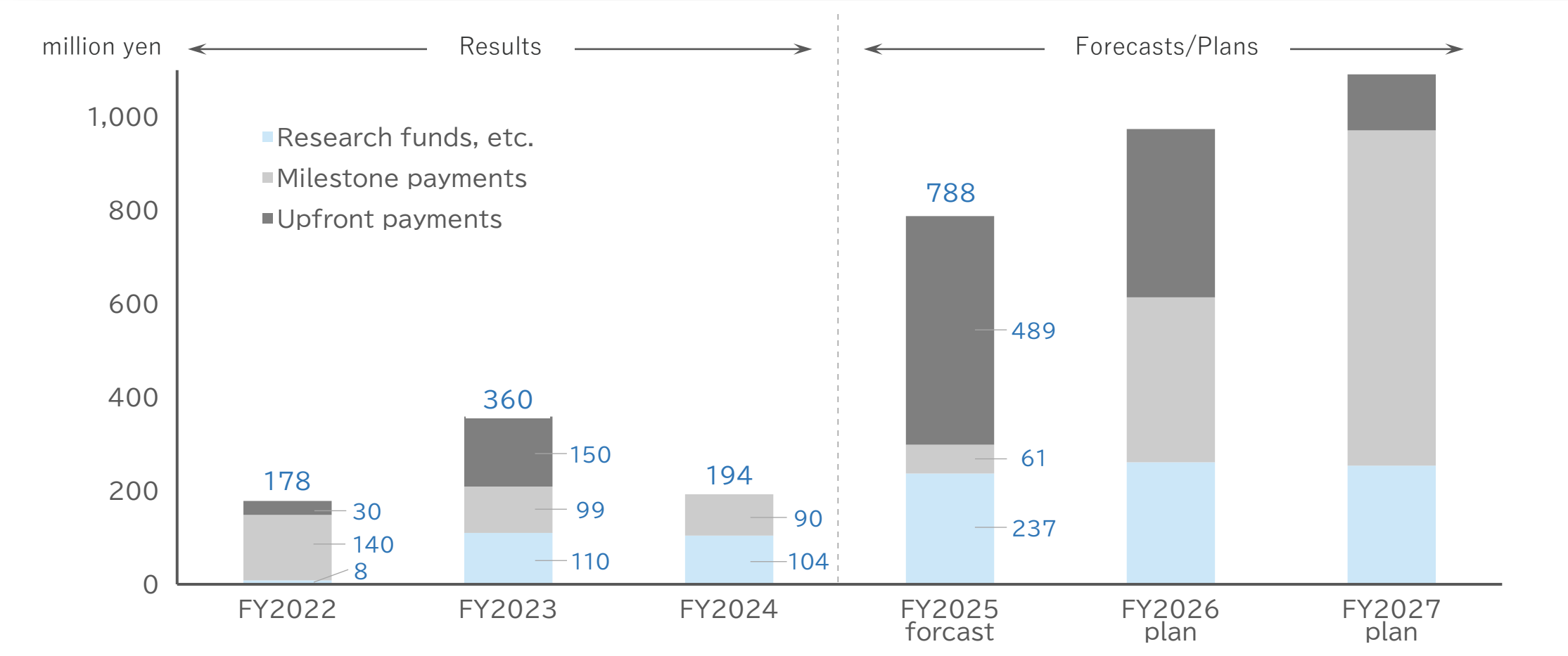


7

Financial Highlights

Track records of business revenue and forecasts/plans from FY2024 onwards

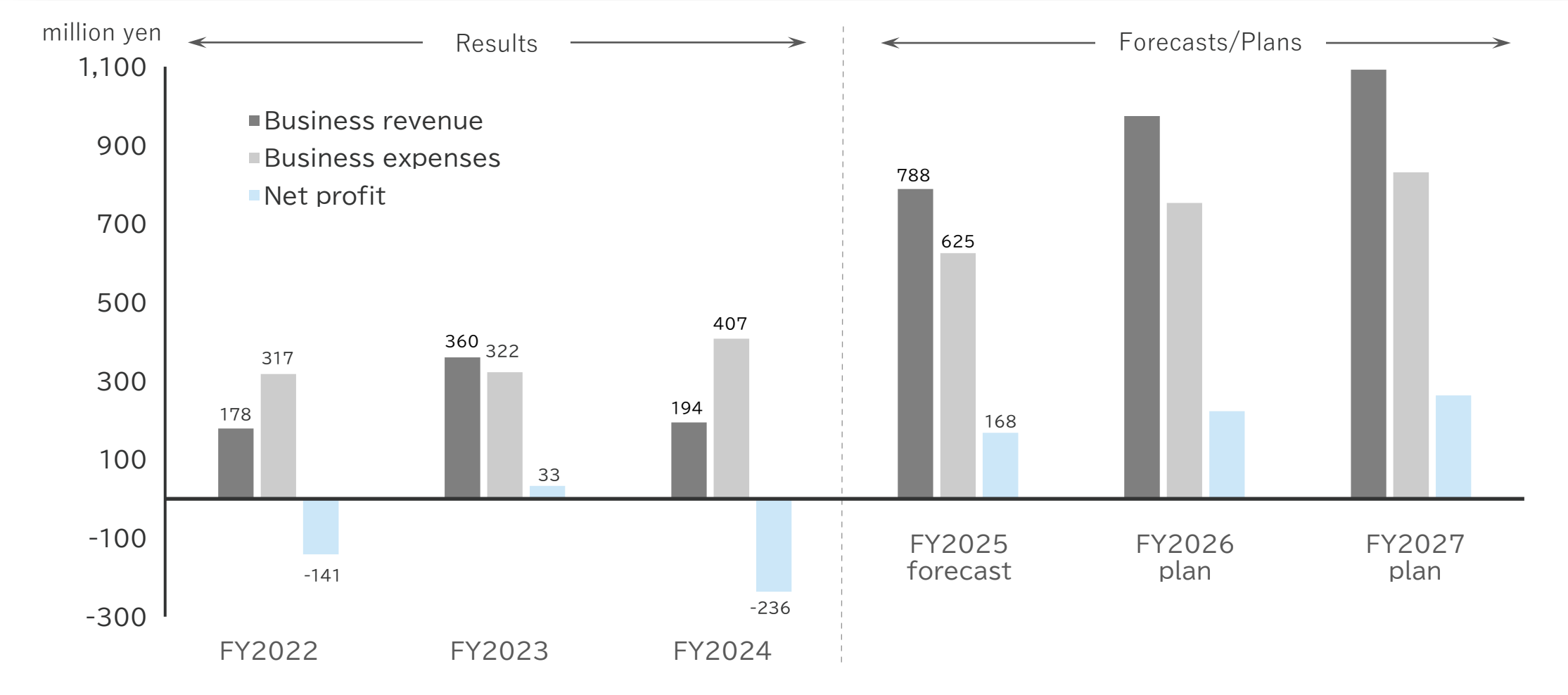
The signing of new contracts expected to be in FY2024 was delayed until FY2025, resulting in a decrease in revenue from upfront payments, etc. From FY2025 onwards, we plan to expand our business revenue by acquiring multiple new contracts each year, as well as steadily progressing joint drug discovery research



Note: The graph for FY2025 to FY2027 is based on the accumulation of expected revenue amount for each customer and project, for which a contract is expected to be concluded. The figures for plans are not disclosed.

Past financial results and forecasts/plans for FY2024 and beyond

Business expenses for FY2024 are expected to rise compared to FY2023 due to increased personnel costs and additional research expenses for pipeline development. During the mid-term management plan period from FY2025 onwards, we will aim to return to profitability by acquiring new contracts and achieving milestones, etc.



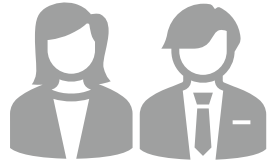
Trends in Quarterly Performance (FY2023-2024)

Quarterly Performance (millions of yen)

Quarter over Quarter	FY2023 Q3	FY2023 Q4	FY2024 Q1	FY2024 Q2	FY2024 Q3	FY2024 Q4
Business revenue	29	81	32	83	49	29
Business expenses	80	83	97	85	104	120
Operating profit (loss)	-51	-2	-65	-1	-54	-91
Non-operating profit (loss)	-1	0	-22	0	0	1
Ordinary profit (loss)	-53	-1	-87	-1	-54	-90
Net profit (loss) for each quarter	-53	-2	-87	-2	-55	-90

Funds raised through the public offering will be used to fuel sustainable growth

We will build an administrative structure appropriate for a publicly listed company while increasing the number of researchers and business development personnel to handle our platform business expansion, including overseas. In addition, we plan to use the funds to finance research equipment and facilities that can withstand the creation of our pipeline in preparation for the shift to a hybrid business model



Manpower plans

Toward transformation into a pharmaceutical company

- Increase research and business development personnel
- Expand administrative structure to withstand the demands of a publicly listed company

430
million yen



Marketing plans

Expanding drug discovery partnerships overseas

- Expand overseas business development activities
- Generate research data directly related to business development
- Business cooperation with ODS

60
million yen



R&D plans

Creation of in-house pipelines

- Prepare for in-house pipeline development
- Schedule to start pre-clinical trials of in-house pipeline in FY2026
- Business cooperation with MGC

390
million yen



Capital expenditure plans

Expansion of facilities and computing capacity to make in-house research robust

- Laboratory expansion and relocation
- Expansion of computing power and AI
- Automation of various processes
- Business cooperation with KDDI

40
million yen



8

Risks of Business

Important business risks and countermeasures

The main risks we recognize as having the potential to significantly impact our ability to achieve growth and execute our business plan are as follows. Please refer to “Business and Other Risks” in the securities report for other risks

Important risks in business operations	Impact	Dealing with risks
Uncertainty of research and development We cannot control the progress of our joint drug discovery research, which is affected by the policies of our partner pharmaceutical companies. We have no track record of proceeding up to the lead compound optimization. These uncertainties in research and development may affect our financial position and business performance.	Occurrence: Low Impact: High	We strive to diversify and mitigate risks by earning a variety of revenue, upfront payments, research support funds, and milestone payments, through conducting multiple drug discovery research projects with partners.
Joint drug discovery research agreements with pharmaceutical companies If joint drug discovery research agreements with partners are terminated due to factors beyond our control, such as changes in the business environment or partner management policies, or if research is suspended, discontinued, or delayed, our business strategy and plans may change, potentially impacting on our financial position and business performance.	Occurrence: Low Impact: Low	We strive to diversify and mitigate risks by earning a variety of revenue, upfront payments, research support funds, and milestone payments, through conducting multiple drug discovery research projects with partners.
Competition with other companies in the biotech industry We provide a one-stop platform for mRNA-targeted small molecule drug discovery and particularly have strengths in target identification. The emergence of competing technologies from other companies could affect our financial position and business performance.	Occurrence: Low Impact: Medium	We will continue to enhance the capabilities of our drug discovery platform by developing new technologies.

Important business risks and countermeasures

The main risks we recognize as having the potential to significantly impact our ability to achieve growth and execute our business plan are as follows. Please refer to “Business and Other Risks” in the securities report for other risks

Important risks in business operations	Impact	Dealing with risks
Filing and acquisition of intellectual property rights We are filing and acquiring necessary intellectual property rights, such as patents, for our business operations. However, not all applications may be approved, and even registered rights could be invalidated due to objections or invalidation trials.	Occurrence: Low Impact: Low	We are working with patent attorneys and lawyers in specialized fields to mitigate risks.
Growth potential for mRNA-targeted small molecule drug market Supposing that the market for mRNA-targeted small molecule drugs does not expand as expected due to factors like emerging toxicity risks or shifts in market positioning caused by next-generation new drugs, we may face difficulties extending partnerships, potentially impacting our financial position and business performance.	Occurrence: Low Impact: Low	Although we expect the relevant market to grow continuously, we will regularly collect information on industry trends and adjust our business operations in response to changes in the business environment
Matters related to cash flow Our company's policy is to strengthen its financial base by raising funds at appropriate times as necessary. If we fail to raise funds when needed, or if the returns are smaller than the investment, it would affect our financial position and business performance.	Occurrence: Low Impact: Low	We plan to strengthen the financial base by raising funds at appropriate times as necessary, considering the necessity of our in-house drug discovery research and the status of future contract conclusion.



Appendix

Glossary No.1

Term	Description
Antibody drug	A collective term for drugs that are intended to produce a therapeutic effect by administering an "antibody" into the body. Because they can act on the target molecule with pinpoint accuracy, they are expected to have a high level of efficacy and reduced side effects. On the other hand, due to their complex manufacturing and difficulty in controlling their quality, antibody drugs have high manufacturing costs and are sold at a high price. Like nucleic acid drugs, they are currently administered only by injection.
ASO	A category of nucleic acid drugs that bind to mRNA and primarily function to control protein synthesis (translation). Various chemical modifications can be introduced to add stability, function, etc. to ASOs.
BLI	Biolayer interferometry. A method for measuring thermodynamic interactions. It uses a device that measures the interaction between biomolecules immobilized onto a sensor chip and molecules in a solution. We use this method to measure the interaction between RNA and small molecule compounds, such as hit compounds obtained through screening, by dipping the sensor chip with immobilized RNA into solutions containing small molecules. Not only can it be performed at high speed, but it also requires minimal amounts of sample.
DNA	Among nucleic acids (biological macromolecules consisting of nucleotides comprised of base, sugar, and phosphoric acid), its sugar portion is composed of deoxyribose, and therefore, it is also called deoxyribonucleic acid. It is a biomacromolecule that is responsible for the transfer of genetic information in almost all living organisms on the earth.
Drug candidate compound	Compounds that have been further improved from lead compounds through chemical synthesis and are the final products of our drug discovery research steps. After confirming the efficacy and safety of a drug candidate compound in non-clinical studies in animals under international standards, the compound is finally tested in human subjects (clinical studies). After submitting the results of the clinical trials to the regulatory authorities, the drug is approved through a review process and becomes a pharmaceutical product.
Hit compounds	A term used in drug discovery. In this document, we refer to active compounds discovered in the initial screening of drug discovery stage as hit compounds.
In silico	Used in biology in analogy to in vivo and in vitro and means "using a computer." In other words, it refers to research based on physiological conditions by quantifying the structure of biomolecules, including genomes, through computer-based calculations.
ITC	Isothermal titration calorimetry. One of the methods used to measure thermodynamics. It uses an instrument to measure the minute changes in heat quantity that occur when molecules bind to each other and to analyze interactions. In our company, we use this method to measure the interaction between RNA and small molecule compounds such as hit compounds obtained through screening. In general, the interaction values obtained are said to be more accurate than those of other methods, but it has the disadvantage of taking a long time to conduct the measurements and requires a large amount of sample.
Lead compound	A term used in drug discovery. In this document, a lead compound is a compound that comes after the hit compound. It is a compound that has been modified by chemical synthesis based on the hit compound and shows better physical properties than the hit compound, such as its activity being confirmed in animals, etc. In addition, the lead compound is used as a starting point for further improvement by chemical synthesis to improve physical properties such as activity, solubility, toxicity, and the ability of the compound to be absorbed when taken as a drug (pharmacokinetics). However, the definition of a lead compound may vary among pharmaceutical companies.

Glossary No.2

Term	Description
Methods for measuring thermodynamics	Methods for monitoring chemical or binding reactions that occur when a compound is introduced to a target molecule by means of isothermal titration calorimetry (ITC) or other methods. Since heat is released or absorbed when molecules bind to each other, the binding between molecules can be quantitatively analyzed by observing the change in calorimetric quantities.
mRNA	Transfers the genetic information, the DNA sequence, and transmits the information for protein synthesis. The mRNA is the blueprint for protein synthesis in the cell, and there is a separate mRNA for each protein.
NCBI	National Center for Biotechnology Information. A public institution established as a division of the National Library of Medicine under the U.S. National Institutes of Health. Since the most reliable genetic information and other data are stored there, we mainly obtain mRNA sequence information for our mRNAs from the NCBI database.
NMR	Nuclear magnetic resonance. One of the spectroscopic methods. A method to obtain structural information on a substance by exposing an atom's nucleus to an external electromagnetic wave in a magnetic field and observing the phenomenon of absorption of a specific electromagnetic wave (resonance phenomenon). In addition to the obtaining RNA secondary structure information, we also use this method to analyze the binding of small-molecule compounds, such as hit compounds to RNA, and the three-dimensional structure of RNA.
Nucleic acid drugs	Drugs that utilize the nucleic acid itself, a substance that controls genetic information, such as DNA and RNA, and can target mRNA and other substances that conventional protein-targeted small molecule drugs and antibody drugs cannot. The molecular weight of nucleic acids is between that of small molecule drugs and antibody drugs, and they are also called medium-sized drugs. Since their commercial manufacturing methods are still in the process of being established, their production costs are even higher than those of antibody drugs, which are said to be expensive. Like antibody drugs, they are mainly administered by injection.
Pipeline	Drug candidate compounds (new drug candidates) in the development stage, including non-clinical and clinical trials, are referred to as pipeline in our company and are distinguished from compounds for projects in the pre-clinical drug discovery research stage
qFRET	Quantitative fluorescence resonance energy transfer. A research method that adds a quantitative dimension to the fluorescence resonance energy transfer method by integrating our proprietary experimental protocols, experimental equipment, and data analysis methods.
Quantum chemistry	A branch of physical chemistry. It is a field of study that theoretically explores molecular structures, physical properties, and reactivity by numerically calculating the behavior of molecules, atoms, and their constituent electrons based on fundamental theories such as Schrodinger's equation.
Research and development (R&D)	A series of processes leading to the launch of a new drug product on the market. Of these, research (drug discovery research and basic research) is the stage up to the acquisition of a drug candidate compound—from "target identification," "screening," "hit validation," and "lead optimization,"—for which we can provide technology through the ibVIS® platform. On the other hand, development is the entire process after the acquisition of a drug candidate compound—from non-clinical studies, clinical studies, regulatory filings, and regulatory approvals.

Glossary No.3

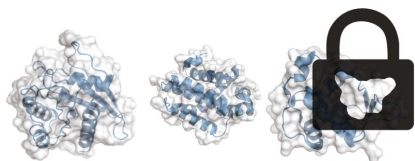
Term	Description
Ribosome	A large RNA-protein complex consisting of several RNA molecules and about 50 proteins. It is divided into two parts, large and small, called 50S subunit and 30S subunit, respectively. It exists in the cells of all living organisms and functions as a machine that synthesizes (translates) proteins by reading the genetic information transcribed in mRNA.
RNA	Among nucleic acids (biological macromolecules consisting of many nucleotides comprised of bases, sugars, and phosphates; DNA is another type of nucleic acid), its sugar portion consists of a ribose, and therefore, it is also called ribonucleic acid. In living organisms, ribonucleic acids are involved in many biological phenomena, such as the transmission of genetic information. It can be classified into messenger RNA (mRNA), which transmits genetic information, transfer RNA (tRNA), which carries amino acids, the raw material for proteins, and ribosomal RNA (rRNA), which makes up ribosomes.
Screening	An experimental method for selecting compounds that meet certain conditions from a large number of compound groups (generally, a chemical library consisting of tens of thousands of compounds or more).
Small molecule drugs	Drugs that generally have a molecular weight of 500 or less. They can be administered in a variety of ways, such as in the form of a pill or a patch. Since they are manufactured by chemical synthesis, controlling their quality is easy, and their commercial manufacturing methods are well established, making them significantly less expensive than antibody and nucleic acid drugs. Therefore, it is the most sold type of drug and accounts for about half of the drug market.
Spectroscopic method	A research method for investigating the quantitative or physical properties of an object by obtaining spectra (components of measurement results arranged according to their size for easy analysis) that show the intensity of physically observed quantities as a function of frequency, energy, time, and other factors. In Japanese, the kanji character for "light" is used, but spectroscopic methods do not necessarily only involve measurements that use light.
Statistical mechanics	Also referred to as statistical physics. A field of physics that applies mechanical and electromagnetic laws and probability theory to the motion of the many particles that make up matter and discusses the macroscopic properties of matter by means of statistical averaging laws. We have found that these theories of statistical mechanics can be applied to RNA structural analysis, and we are applying them to drug discovery.
Synthesis of compounds	The synthesis of many small molecule compounds with the aim of creating small molecule drugs. To be specific, starting from small molecule compounds that have been successfully identified in screening, numerous small molecule compounds with similar structures are designed and organically synthesized to meet the desired properties (improvement of activity, pharmacokinetics, reduction of toxicity, etc.). The newly synthesized small molecule compounds are subjected to various tests to obtain a more suitable small molecule compound, and the synthesis continues with that compound as the starting point. This cycle continues until a compound with a sufficient profile as a small molecule drug is obtained.
Thermodynamics	A branch of physics that studies the thermal properties of matter from a macroscopic standpoint. It is a theory that deals with the macroscopic properties of entire systems. It is not often considered applicable to biology, which is a complex system. We have found that these thermodynamic theories can be applied to RNA structural analysis, and we are applying them to drug discovery.

More targets for small molecule drugs provided by mRNAs than proteins

Small molecule drug discovery involves a series of steps to search for “locks” on drug targets and find “keys” that fit perfectly into the locks. Our greatest strength is our ability to find “locks” on most mRNAs using in silico (computational) RNA structural analysis. We have also established our own quantitative screening method for finding “keys” that fit into the locks on mRNAs

Protein-targeted drug discovery

Target identification



! Problem : Only limited number of proteins with locks remain (depletion of drug targets)

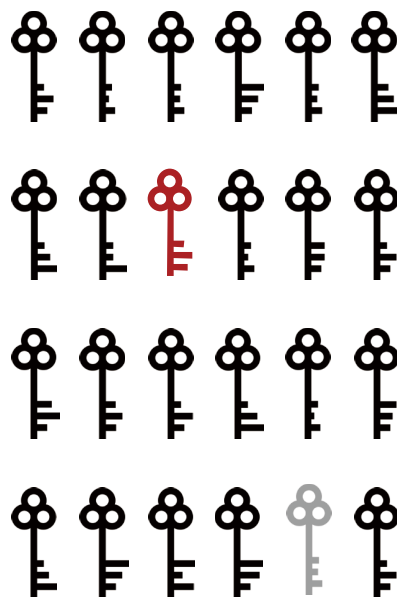
Screening₁



¹A process of sifting through a large number of compounds and select compounds that meet a certain criteria, i.e., a process to find candidate keys for a lock

²Compounds that meet the screening criteria (candidate keys)

Small molecule compound collection*



*The collection of small molecule compounds used in screening is called a chemical library

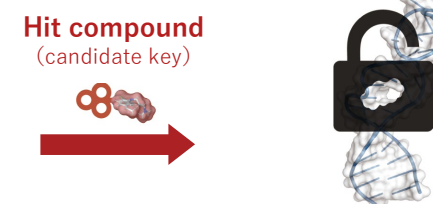
¹⁴Si mRNA-targeted drug discovery

Target identification



Our technology : In silico structure analysis of mRNA partial structures. We can find “locks,” where “keys” can fit into, on most mRNAs

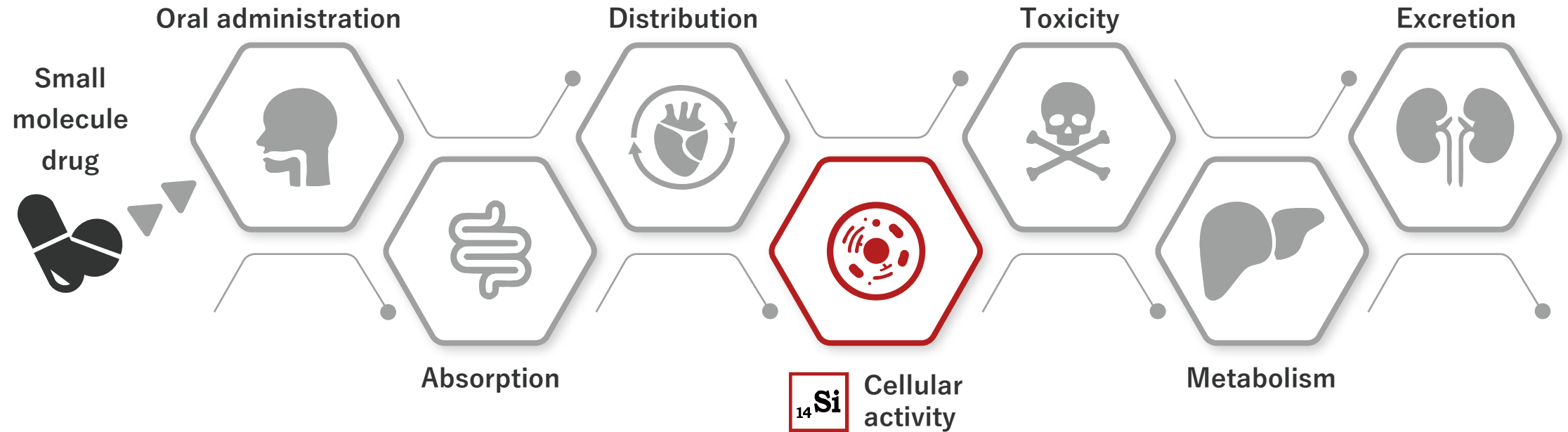
Screening



Our technology: Quantitative screening method to find “keys” that fit into the “locks” on mRNAs

Does the mRNA-targeted small molecule exert its activity within cells?

In drug R&D, it is important to consider not only the main action of a drug (or drug candidate) but also other aspects, such as absorption, distribution, metabolism, excretion, and safety. Despite being a novel approach, the only aspect to be considered that is unique to mRNA-targeted small molecule drug discovery is the main action or cellular activity



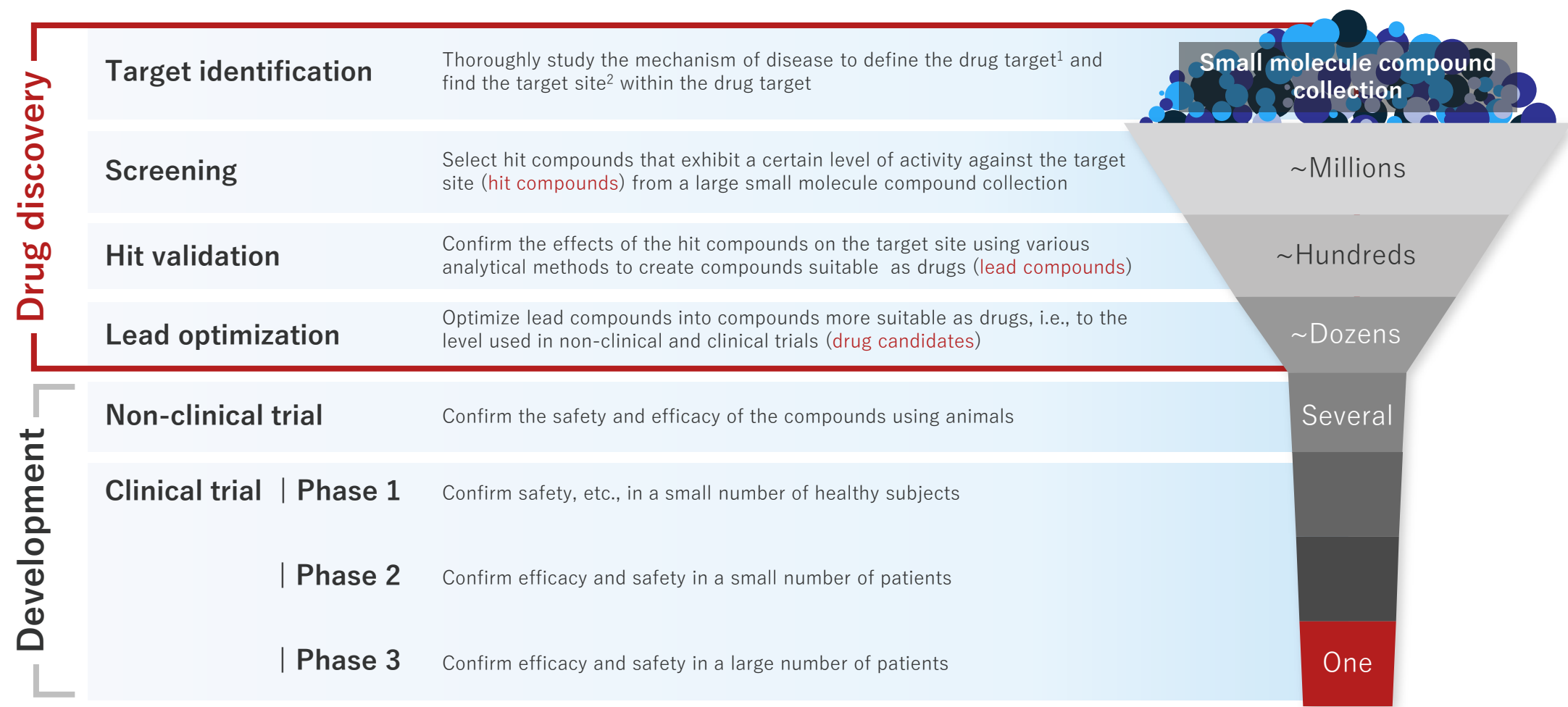
Specific to small molecule drug discovery and common between protein- and mRNA-targeted approaches



Requires the VIS platform technology and is unique to mRNA-targeted small molecule drug discovery

Focus on drug discovery research for mRNA-targeted small molecule drugs

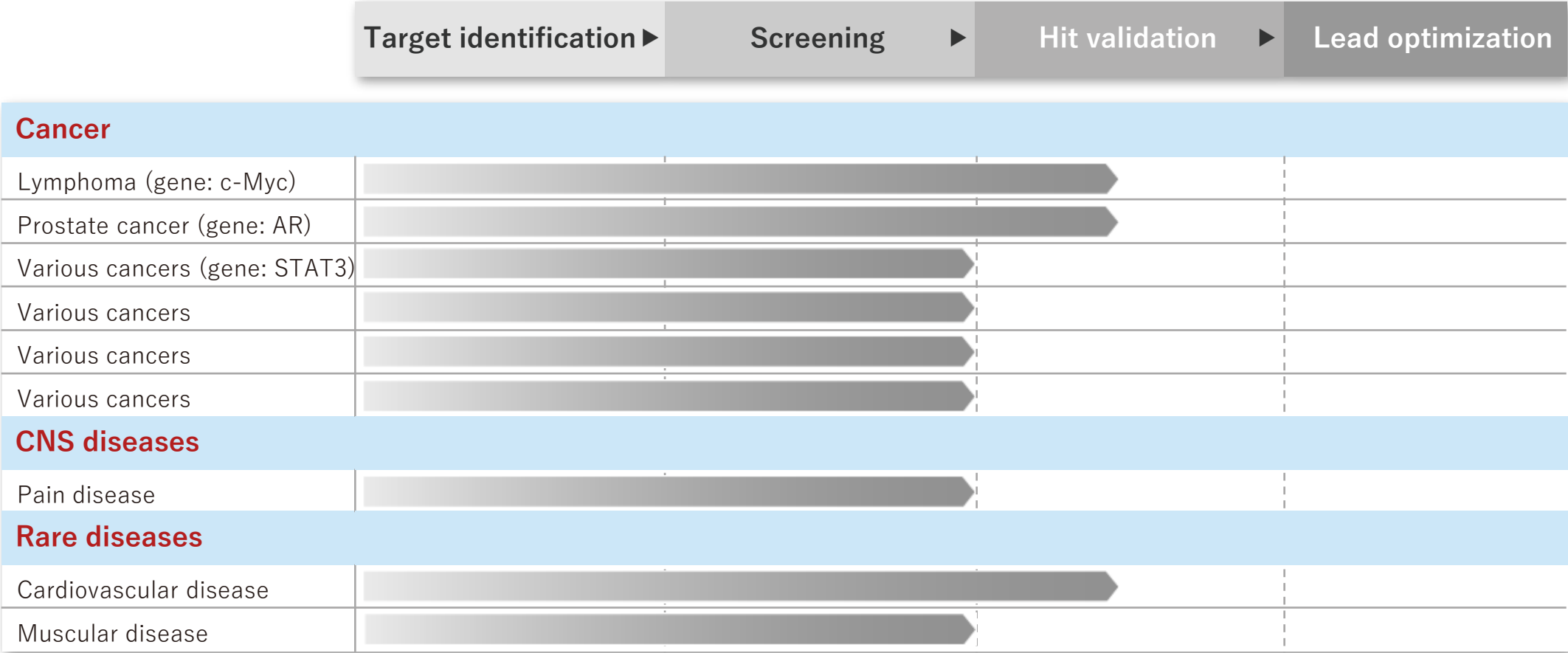
Our drug discovery research capabilities allow us to discover small molecules, which exert their activity on mRNAs within cells, in a manner similar to general drug discovery research



¹Disease-related proteins and their blueprints, mRNAs, are the drug targets. In the drug industry, they are generally referred to as "target molecules."
²After determining the drug target, it is necessary to determine which part (structure) of the overall drug target is to be targeted by the drug. In our case, the site (structure) to be targeted by a small molecule drug is called a "target structure."

Small molecule in-house pipeline candidates for future maximization of corporate value

We are preparing our in-house small molecule projects that will serve as the basis for our future hybrid-type business, with a focus on the oncology field where the medical needs are high. We plan to create our own pipeline from projects capable of generating sales of 20 billion yen or more per year



Note: Above projects are originated from collaborative researches with partners, however, currently (as of December 31, 2024) all projects are suspended. If we resume the projects, to secure our freedom of business operation, we will conduct screening again with our own chemical library.

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In principle, this document will be updated and disclosed annually around the time announcing the end-of-term financial results.

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