INTRODUCTION

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Our Philosophy







Our Philosophy

Helping People Lead Healthier, Happier Lives



Our Promise

A New Way of Life

Delivering meaningful value to create a better future for every patient

Guided by our philosophy of "Helping People Lead Healthier, Happier Lives," Nippon Shinyaku has remained firmly committed to addressing the challenges of intractable and rare diseases —areas where treatment options are limited.
The driving force behind this commitment is our unwavering determination to create a future allowing patients and their families to live with a sense of security and genuine hope.
We continue our bold, sincere efforts to break new ground and address unmet medical needs, no matter how rare a disease may be, as we strive to create new ways of life for every patient around the world.





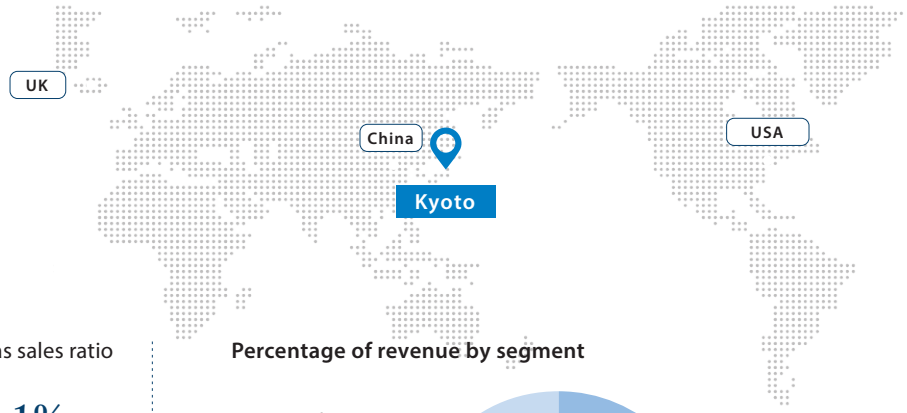
About Nippon Shinyaku (Figures for FY2025 or as of March 31, 2026)

From Our Beginnings in Kyoto to a Pharmaceutical Company Tackling Intractable and Rare Diseases

Revenue
¥170.7billion
Increase of 6.6% year-on-year

Operating profit
¥35.4billion
Increase of 0.1% year-on-year

ROIC
13.1%
Decrease of 3.9 pt year-on-year



R&D expenses
¥36.7billion
Increase of 6.9% year-on-year

Percentage of R&D investment allocated to intractable and rare diseases*1
70.7%
Increase of 7.0 pt year-on-year

Percentage of sales*2 from therapeutic agents for intractable and rare diseases*1
74.2%
Increase of 1.8 pt year-on-year

Percentage of sales from new products*3
83.0%
Increase of 10.8 pt year-on-year

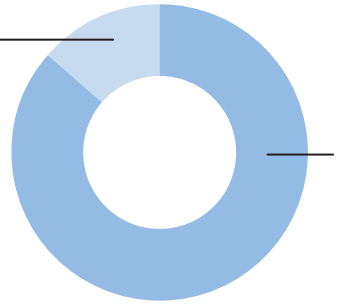
Number of development pipelines*4
24
Increase of 2 year-on-year

Percentage of intractable and rare diseases in the pipeline
87.5%
Increase of 1.1 pt year-on-year

Overseas sales ratio
45.1%
Decrease of 2.2 pt year-on-year

Number of countries where products are sold
76
Increase of 1 year-on-year

Percentage of revenue by segment



Functional Food Business
13%
Increase of 3.3% year-on-year

Pharmaceuticals Business
87%
Increase of 7.1% year-on-year

Percentage of research personnel
29.8%
Increase of 0.3 pt year-on-year

Percentage of women in management positions
15.5%
Increase of 1.1 pt year-on-year

Uptake rate for childcare leave (male)
75.0%
Decrease of 1.2 pt year-on-year

Digital transformation promotion talent
1,043
Increase of 256 year-on-year

External evaluation of human resources and digital transformation

- Health & Productivity Management Outstanding Organization (White 500: top 500 large enterprises)
- KENKO Investment for Health Stocks
- "Eruboshi" Certification
- "Kurumin" Certification
- Digital Transformation Certification

*1 Including hematology *2 Including income from industrial property rights, etc.
*3 Percentage of pharmaceutical sales (excluding income from industrial property rights, etc.) accounted for by original drugs on the NHI price listing within the past 15 years and for which no generic drugs are available
*4 The aggregation criteria were revised in FY2025, and the previous fiscal year's results were recalculated accordingly

A Century of Progress Built on the Challenge of Drug Discovery

Since its founding in 1919, Nippon Shinyaku has carried on the founding spirit of "making Japanese medicines with Japanese hands," working to create new drugs that meet the hopes of patients.
In recent years, we have designated intractable and rare diseases as priority areas and have continued to strive to provide patients with new treatment options.

Early years

The founding spirit that inspired our commitment to taking on challenges

In 1911, at a time when Japan had little choice but to rely on imports for many of its pharmaceuticals, our founder, Hisomu Ichinose, undertook the task of producing Santonin vermicide domestically.
After 15 years of research and quality improvements, he succeeded in establishing domestic production, which subsequently contributed significantly to the decline in roundworm infection rates in Japan.
This pioneering spirit lives on in our drug discovery efforts today.



Founder
Hisomu Ichinose

2000s

Establishing an original drug discovery platform

The company began researching nucleic acid drugs in the 1990s and established proprietary RNA synthesis technology in 2007.
We also established drug discovery technologies that enable us to target diseases that have been difficult to treat with conventional small molecule drugs, and built an R&D foundation to support efforts to address intractable and rare diseases.
This technology continues to contribute to the development of nucleic acid drugs today.

2010s

Focusing on intractable and rare diseases

We identified intractable and rare diseases, which affect small patient populations and have limited effective treatment options, as a growth area and therefore increased our investment in R&D.
Uptravi, our in-house discovered treatment for pulmonary arterial hypertension, has evolved into a global product sold in 76 countries.
We established a high level of expertise in intractable and rare diseases, which has also contributed significantly to enhancing our business foundation.

2020s

Taking on the global challenge in intractable and rare diseases

We launched Viltepso, the first nucleic acid drug developed in Japan, in the U.S. market in 2020.
In addition to establishing its own marketing infrastructure in the U.S., the company opened Innovation Research Partnering (IRP) in Cambridge in 2023 to accelerate its global expansion.
Currently, 87.5% of our development pipeline consists of treatments for intractable and rare diseases, with the goal of providing new treatment options to patients around the world.

2004
Trisenox, a treatment for relapsed or refractory acute promyelocytic leukemia

2009
Adcirca, a treatment for pulmonary arterial hypertension

2016
Uptravi, a treatment for pulmonary arterial hypertension



2020
Launched Viltepso, Japan's first nucleic acid drug



2023
Established IRP drug discovery center in the U.S.

2026
Elzonris, a treatment for blastic plasmacytoid dendritic cell neoplasm

Trends in revenue and operating profit

1919
Nippon Shinyaku founded

1997
Discovery Research Laboratories in Tsukuba completed
Full-scale research into nucleic acid drugs

2005
Amnolake, a treatment for relapsed or refractory acute promyelocytic leukemia

2007
Established world's longest RNA synthesis technology

2011
Vidaza, a treatment for myelodysplastic syndromes



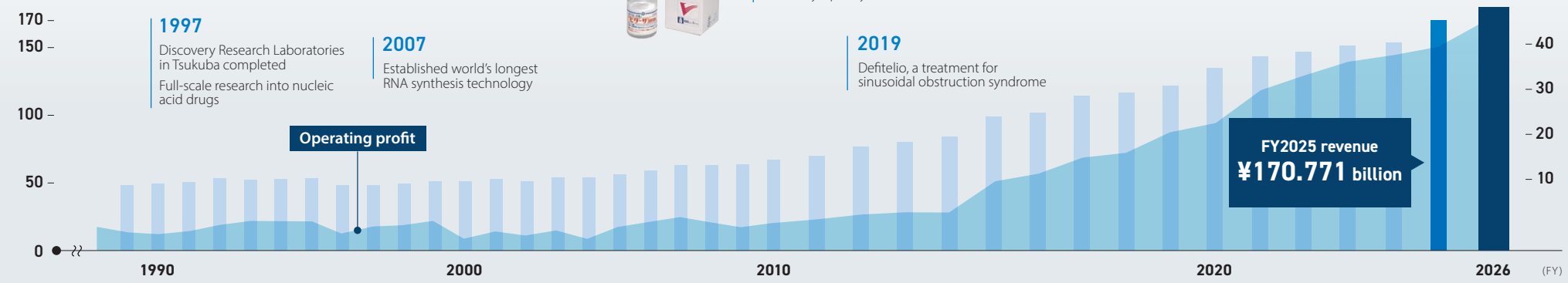
2018
Gazyva, a treatment for CD20-positive follicular lymphoma and CD20-positive chronic lymphocytic leukemia

2019
Defitelio, a treatment for sinusoidal obstruction syndrome

2022
Fintepla, an anti-epileptic treatment

2024
Jaypirca, a treatment for mantle cell lymphoma





Addressing the Challenge of Intractable and Rare Diseases

Addressing Overlooked Diseases Head-On

We will continue to open up new possibilities in areas where treatment options are still limited.

Due to the small number of patients and the challenges inherent in conducting research, there are certain diseases where sufficient treatment options have yet to be established.
Leveraging our strengths in drug discovery platforms, including nucleic acid drugs, Nippon Shinyaku has continued to confront intractable and rare diseases for which treatment options have historically been limited. As we gain deeper insights into each individual disease, we will steadily build on new possibilities and directly address the pressing needs of patients, their families, and healthcare professionals, thereby creating new treatment options in areas with significant unmet medical needs.

Percentage of intractable and rare diseases in the pipeline

87.5%

Percentage of R&D investment allocated to intractable and rare diseases

70.7%

Therapeutic areas

- Hematology
- Immunology
- Neuromuscular
- Pulmonary hypertension



Message from the President



Nippon Shinyaku remains steadfast in its commitment to intractable and rare diseases.

Representative Director, President

Toru Nakai

Nippon Shinyaku's journey and unwavering commitment

Since its founding in 1919, Nippon Shinyaku has remained committed to discovering and delivering, through its own efforts the medicines that patients truly need. The intent of our founder, Hisomu Ichinose, to "make Japanese medicines with Japanese hands" remains at the core of our decision-making today, even though the business environment has changed dramatically.

Modern medicine has progressed in significant ways, expanding the range of treatment options available for countless diseases. On the other hand, there are still many diseases for which we continue to lack effective treatments. In the field of intractable and rare diseases in particular, although each disease affects relatively few patients, the range of diseases is vast, with adequate treatment options still lacking for many. That is exactly why I believe that addressing intractable and rare diseases is our company's primary mission. This approach is clearly reflected in our R&D strategy, with approximately 80% of our current development pipeline focused on intractable and rare diseases. This is no coincidence, but rather the direct result of decisions made consistently over many years to allocate management resources based on patient needs, refine our expertise, and improve both our speed and the likelihood of success. It is this sustained focus and steady accumulation of expertise that have shaped the distinctive strengths and capabilities that make Nippon Shinyaku what it is today.

Message from the President

One example of an initiative that has helped us establish our originality is our R&D of nucleic acid drugs. We recognized the potential of these medicines even before the technology was established and continued our research while steadily investing in both our research capabilities and human resources. Our R&D in nucleic acid drugs has grown beyond the mere accumulation of technology, shaping Nippon Shinyaku's corporate philosophy itself. Nippon Shinyaku aspires to be a company that provides high-quality, unique pharmaceuticals that patients suffering from illness and their families truly need. This also means expanding the range of treatment options as part of our business activities and continuing to contribute to the resolution of societal challenges. It is this commitment that makes Nippon Shinyaku an indispensable company in the global healthcare field.

Our business environment will continue to evolve in response to structural changes in the Japanese market, rapid technological innovation, and intensifying global competition. While intractable and rare diseases carry great social significance, the process from development to market launch and widespread distribution of treatments to those who need them also presents numerous challenges. Despite this, I believe this is precisely where Nippon Shinyaku can best demonstrate its distinctive strengths. Based on these foundational beliefs, we have defined our vision for 2035 and are taking steps to bring it to fruition.

Our vision for 2035 and progress of our Medium-Term Management Plan

Our focus on intractable and rare diseases is not only a social mission for us, but also a core element of a sound business strategy that contributes to improving capital efficiency. Our vision for 2035 is to be a global healthcare company from Kyoto creating various types of new ways of life for each person around the world. To achieve this and ensure sustainable growth, we formulated the 7th Five-Year Medium-Term Management Plan "For Global Growth Beyond the Cliff," to map out our path from FY2024 to FY2028.

Under this Medium-Term Management Plan, we view Uptravi's patent cliff as a critical turning point and have identified three key themes: fostering growth drivers to replace Uptravi, expanding global development, and continuous pipeline expansion. To ensure steady progress on these three key themes, we will rigorously allocate resources and manage costs in line with the priorities set out in our management strategies, while managing the capital efficiency of each business using ROIC to secure returns that exceed the cost of capital. Under this policy, in FY2025, we achieved a record-high consolidated revenue of ¥170.7 billion and operating profit of ¥35.4 billion, marking growth in both revenue and earnings, with ROIC at 13.1%.

In our Pharmaceutical Business, we are steadily working to forge new pillars that will support future growth. In Japan, we worked to expand our value in the pulmonary hypertension field, including with the launch of the pediatric formulation of Uptravi. In hematology, we expanded our treatment options with an additional indication for the antineoplastic agent Jaypirca, broader use of Gazyva in combination therapies, and the launch of Elzonris I.V. injection for the treatment of blastic plasmacytoid dendritic cell neoplasm. In the field of urology, we bolstered our portfolio by acquiring the marketing rights to the prostate cancer treatment Erleada.

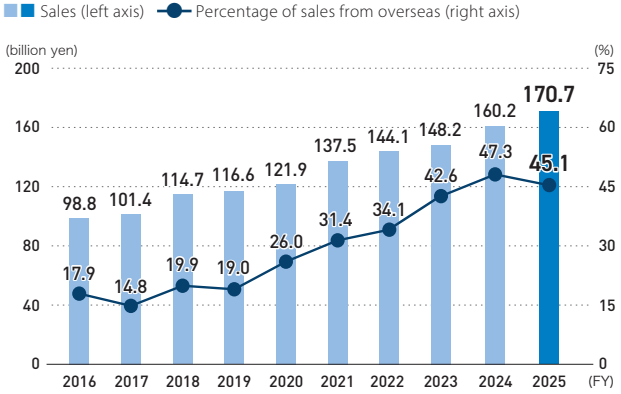
In addition to the above, for our portfolio of new products, including Viltepso for the treatment of Duchenne muscular dystrophy (DMD) and Fintepla for the treatment of epileptic seizures in the pediatric field, we are expanding omnichannel sales activities that combine MR and digital channels to drive the early market uptake of these products. In our global operations, we are steadily expanding our next-generation pipeline with initiatives such as the in-licensing of RGX-121/RGX-111 for the treatment of mucopolysaccharidosis and an option agreement for Tadekinig alfa.

At the same time, uncertainty in the business environment surrounding pharmaceuticals is on the rise, due to factors such as Japan's domestic drug pricing system and U.S. pricing policies, including the Most-Favored-Nation (MFN) framework. In the course of advancing our R&D, there may also be times when progress does not proceed as planned due to factors such as regulatory changes or delays in development. For this very reason, we intend to place even greater emphasis on our choice of research topics, resource allocation, and the speed of commercialization, while steadily developing future growth drivers in FY2026. What matters most is to establish a structure in which multiple growth drivers can emerge on an ongoing basis, regardless of performance in any given fiscal year. To achieve this, we need to maintain a rigorous focus on our priority areas while designing clinical trial plans with an emphasis on success rates and speedy action, exercising sound judgment in in-licensing and partnerships, and strengthening our ability to deliver treatments to patients by integrating marketing, reimbursement, patient support, and supply.

At the same time, to accelerate decision-making and execution during R&D, our goal is to further strengthen project prioritization and progress management, while ensuring greater involvement from senior management as necessary. Furthermore, we will expand the use of digital technologies and AI to enhance the speed and quality of our trial-and-error process by identifying promising research topics, streamlining our development processes, and improving company-wide productivity overall.

In terms of profitability, we expect profits to increase progressively from FY2026 onward. In FY2028, the final year of our Medium-Term Management Plan, we will solidify a growth trajectory beyond the patent cliff by achieving our targets of ¥230.0 billion in revenue, ¥30.0 billion in operating profit, and ROIC of 9% or higher, thereby laying a solid foundation for realizing our vision for 2035. Even if faced with short-term fluctuations, we will remain firmly committed to our core strategic focus on intractable and rare diseases. By steadily delivering results and creating social value to enhance corporate value, we will achieve sustainable growth and continue to earn the trust of our stakeholders.

Sales / Percentage of sales from overseas



Message from the President

R&D strategy

R&D drives value creation at Nippon Shinyaku. We have focused our R&D efforts primarily on intractable and rare diseases, with a strong commitment to bringing unique new drugs to market as quickly as possible. While this field presents challenges in designing clinical trials and securing sufficient numbers of patients, expanding treatment options is immensely important, given the pressing needs of patients and their families. Leveraging regulatory and evaluation insights can accelerate development and improve the probability of success.

Our strength lies in our expertise, underpinned by a solid understanding of diseases, a strong network of healthcare professionals, and a proven track record with our products. For example, we have been advancing the development and commercialization of Viltepso in the field of DMD, building on our accumulated research expertise in nucleic acid drugs. The insights gained from our experience are being applied to identifying promising new research topics and the design of development programs aimed at maximizing value after product introduction.

In our approach to advancing R&D, we place particular emphasis on the combination of expertise, speed, and probability of success, while taking scale into account. We are prioritizing research topics and optimizing resource allocation. In FY2025, we invested ¥36.7 billion in R&D, concentrating these resources on priority areas, while formulating and advancing clinical trial plans focused on maximizing the probability of success. Another distinctive feature of our R&D is our use of multiple modalities, including small molecules, nucleic acid drugs, and gene therapy, to advance our in-house drug discovery efforts. Given that many intractable and rare diseases are hereditary and therefore particularly well suited to treatment with nucleic acid drugs and gene therapy, we are seeking to differentiate ourselves by developing highly original research topics that combine disease characteristics and mechanisms of action with the strengths of each modality. This is the approach to drug discovery that Nippon Shinyaku strives for: embodying our commitment to thinking through every possibility to overcome difficult challenges while maintaining an unwavering focus on maximizing our probability of success.

In expanding our pipeline, we will optimize our approach by combining in-house drug discovery with in-licensing and partnerships. Our aim with in-licensing is not simply to increase the number of projects. Rather, we intend to pursue opportunities only after carefully ascertaining whether we can maximize their value globally and assume responsibility from development and market launch to subsequent information provision and patient support, while taking our future product portfolio into account. We have expanded our in-licensing and partnerships to a total of 19 companies to date. Going forward, we will focus on the three pillars of in-house drug discovery, in-licensing, and product lifecycle management (PLCM) to continuously expand our pipeline and strengthen our execution capabilities.

Speeding up R&D remains a key management priority. While the number of proposed research topics is increasing, not all of them are progressing smoothly to the development phase. We are therefore accelerating our initiative to identify promising research topics at an early stage and ensure their smooth transition to the development stage. By incorporating data analytics and AI, we are evaluating research topics starting in the discovery stage with their subsequent development in mind. We are also working to strengthen our in-house R&D capabilities by enhancing the process from generating research topics to advancing them into actual development. Furthermore, we view open innovation as a vital means of generating research topics and advancing early-stage discovery. In 2025, we entered into a strategic partnership with Boston Children's Hospital to develop innovative treatments for rare diseases. As part of this partnership, we established a framework for collaborative research based on the submission and selection of research topics.



In collaboration with FRONTEO, Inc., we are leveraging AI-driven drug discovery support services to identify promising therapeutic targets. By incorporating external expertise and technologies in this way, we aim to accelerate the creation of first-in-class new drugs for intractable and rare diseases.

To develop drugs that are truly beneficial to society and patients, it is essential to keep the patient perspective in mind at every stage of R&D. By gaining a deeper understanding of patients' needs and consistently incorporating those insights into drug discovery and development planning, we aim to instill a patient-centered approach throughout our organization. Drawing on the insights gained from these perspectives, we will work to develop new drugs that embody Nippon Shinyaku's unique strengths and originality and bring them to market as soon as possible.

Progress toward expanding global development

Our aim in accelerating our global expansion is to leverage the strengths we have cultivated in the field of intractable and rare diseases to bring new treatment opportunities to more patients across national and regional boundaries. In light of ongoing structural changes in the domestic market, we are also aiming to achieve an overseas sales ratio of at least 50% as a practical strategy for simultaneously expanding growth opportunities and solidifying our business foundation. In the 7th Five-Year Medium-Term Management Plan, the Company has clearly outlined its policy of steadily advancing its global expansion while flexibly utilizing all available means, including in-house marketing, alliances, and M&A.

In FY2025, overseas revenue was ¥77.0 billion, accounting for 45.1% of total revenue, marking steady progress toward our target. Uptravi is also marketed in 76 countries by our partner Johnson & Johnson, contributing to the steady expansion of our global presence. Rather than viewing this ratio as simply an end result, I firmly believe that continuing to strengthen our business capabilities in each region is what will drive our next phase of growth.

Message from the President

In the U.S., we are working to strengthen our comprehensive business capabilities across sales, reimbursement, patient support, and supply, with our subsidiary NS Pharma at the core of these operations. We have strengthened our operations to capitalize on our expertise, experience and the potential of our facilities, thereby evolving into an organization capable of maximizing results. In the DMD field, we will continue to market our internally developed therapy, Viltepso, through our own U.S. commercial organization. At the same time, we are preparing for future opportunities involving CAP-1002, a human allogeneic cardiosphere-derived cell therapy, in-licensed from Capricor Therapeutics. The more we adopt new modalities, the more critical quality assurance, a stable supply, and patient access become to our overall success. In the U.S., we have made it our highest priority to establish an operating model that enables us to continuously launch multiple products.
We largely completed the development of our organizational structure in FY2025, and will now work to steadily strengthen our operational foundation by introducing new products. We will further strengthen our presence in the U.S. by advancing the development of DMD candidates originating from our in-house drug discovery, including NS-089 and NS-050, which are currently in clinical trials, and by eventually bringing RGX-121/RGX-111 and ATSN-101 to market, which are currently being developed by our partners.
In China, with Beijing Nippon Shinyaku Co., Ltd. and Tianjin Nippon Shinyaku Co., Ltd. serving as our bases of operations, we are building on the expertise gained from our existing businesses to develop human resources and strengthen marketing capabilities to ensure the smooth delivery of future in-licensed and in-house discovered products. We are also establishing a patient access framework that takes into account the healthcare system and distribution channels. Despite differing market conditions and regulatory frameworks, we are building an operational structure centered on our local operations to support the sustained expansion of our business. In Europe, we are also taking a phased approach to preparing for future business expansion while considering a range of options, including in-house marketing.
In FY2026, we will further reinforce our organizational structure in the U.S. and advance preparations to introduce new products, while in China and Europe, our initiatives will focus on establishing business foundations tailored to the respective market environments and developing a concrete framework for patient access. By clearly defining our priorities in each region and steadily implementing our initiatives, we will expand the role we play in the field of intractable and rare diseases on a global scale.

Human capital

Nippon Shinyaku's greatest competitive strength is its human resources. The reason we have been able to continually take on the challenges presented by the highly demanding field of intractable and rare diseases is that each and every employee we have is genuinely committed to our patients and has worked across departmental boundaries to deliver results. As part of the 7th Five-Year Medium-Term Management Plan, one of the pillars for reinforcing our management foundation is the promotion of human capital management that allows each employee to grow and diverse human resources to play an active role. We have clearly set out a policy of pursuing growth without increasing our domestic workforce by securing and developing global human resources and transformation leaders who will drive digital transformation, reforming our organizational culture, and improving productivity by leveraging digitalization.
At the beginning of the year, I called on our employees to break free from preconceived notions and drive change from the ground up. As the pace of change in the business environment accelerates, we cannot sustain growth by simply building on conventional ways of thinking. This is particularly true in the field of intractable and rare diseases, where we often face unprecedented challenges and cannot make progress simply by waiting for the right answer. Without fear of failure, we start small, learn, and make continual improvements. I believe that our ability to sustain this cycle as an organization will ultimately determine our growth over the medium to long term.

In recent years, we have seen remarkable advances in digital technologies, including AI. I don't view AI as a replacement for human tasks, but rather as a partner that expands the possibilities for creativity and new challenges. We can use AI to improve the speed and quality of our trial-and-error process and focus on more meaningful value creation.
With this in mind, we will pursue investment in digital infrastructure and human resource development as key priorities of our human capital management. At the same time, dialogue remains the core of our work no matter how far technology advances. It is our connections with one another that inspire people, build trust, and give us the strength to keep taking on new challenges.
With the NS Mind—the attitude and mindset that all employees should aspire to—centered on "Give your all for yourself," "Give your all for others," and "Give your all for society," we will further foster a culture that combines individual initiative with collaboration and firmly establish the strengths of our small, highly capable, and distinctive workforce as an organizational resource.

Final thoughts

Drug development inherently involves uncertainty. There is always the possibility that products in development that are expected to drive future growth may not progress as planned, or that the regulatory and market environments may change. Although we are currently facing litigation surrounding CAP-1002, our mission to provide patients with new treatment options as quickly as possible remains steadfast. To fulfill this mission, we will steadily make the necessary preparations while cooperating closely with all relevant parties.
We are not a company that only chooses paths with favorable prospects. We will pursue sustainable growth while building a business structure capable of continuously generating multiple growth drivers. We will stay fully committed to confronting the challenge of developing pharmaceuticals, with a particular focus on intractable and rare diseases. Our resolve to do so will remain unwavering. Nippon Shinyaku aspires to be a company that continues to create meaningful value for society over the long term, rather than being preoccupied with short-term results.

We will strive to live up to the trust placed in us by all our stakeholders, including patients and their families, healthcare professionals, shareholders, investors, and our employees, as we continue to pursue growth and fulfill our responsibilities well into the future.
I sincerely appreciate your continued understanding and support in the future.

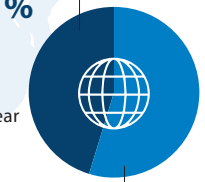
Global Growth Strategy: Accelerating Value Creation Toward 2035

In its 7th Five-Year Medium-Term Management Plan, Nippon Shinyaku has identified "expanding global development" as a key theme.
As we work toward realizing our vision for 2035 as a global healthcare company from Kyoto creating various types of new ways of life for each person around the world, we shall accelerate our growth globally.

Growth Strategy

With a focus on intractable and rare diseases, the Nippon Shinyaku Group is expanding its global business by drawing on both products discovered in-house and cooperation with external partners.
Our in-house drug discovery efforts began with Viltepso, a treatment for Duchenne muscular dystrophy (DMD), and we are now expanding our pipeline in this field with exon-skipping drugs such as NS-089 and NS-050. We are also strengthening our technological foundation by partnering with overseas biotechnology companies to develop new modalities such as gene and cell therapies, while working to diversify our development pipeline.
These initiatives are enabling us to expand our portfolio of products and strengthen our global business foundation.

Breakdown of revenue by region/ Revenue composition ratio



Overseas
45.1%
¥77.099 billion
Increase of 1.8% year on year

Japan
54.9%
¥93.672 billion
Increase of 10.9% year on year



Establishing a Supply and Marketing System

With the goal of improving patient access globally, we are establishing our own marketing organizations in the U.S. and China.While exploring opportunities to expand our business in Europe, we are also considering entry into other regions in cooperation with partner companies, with the goal of delivering our pharmaceuticals to patients around the world.
At the same time, we are enhancing our reliability assurance system to meet regulatory requirements in various countries and strengthening our supply chain, while developing distribution systems capable of handling new modalities such as gene therapy drugs and cell therapy products.
In addition, our Kyoto headquarters and overseas Group companies will cooperate to establish a framework for the integrated management of all functions from research to marketing from a global perspective, thereby accelerating our global expansion.


Innovation Research Partnering office

Development Progress and Challenges

We are pursuing open innovation centered on cooperation with external partners in order to boost our R&D capabilities. With the goal of developing new therapeutic agents for intractable and rare diseases, we are reinforcing our cooperation with academic institutions and biotech startups around the world. As a core function of this initiative, we have established Innovation Research Partnering, based in Cambridge, Massachusetts, to create a framework for translating cutting-edge research findings into drug discovery leads at an early stage. For example, as part of our initiative with Boston Children's Hospital, we call on the hospital's researchers for research topics with the potential to lead to the development of new drugs, select the most promising among them, and then go on to pursue joint research. By combining world-class research capabilities with our expertise in drug discovery, we aim to accelerate our in-house drug discovery efforts and enhance our R&D portfolio, thereby expanding our future pipeline.

Meanwhile, in the context of global pharmaceutical development, as we establish a development framework that leverages cooperation with external partners, we recognize that various challenges may arise from differences in development and distribution processes, partnership arrangements, and business environments across countries.
We are addressing these issues calmly and methodically by engaging in dialogue with the parties concerned and consulting with regulatory agencies. With regard to CAP-1002, a cell therapy product for DMD, we will continue discussions with our business partners and other concerned parties as we proceed with its development and determine our future course of action. We are also considering our next steps for the gene therapies RGX-121 and RGX-111 in light of the progress being made by our partner companies.
Our top priority is to ensure that patients have access to treatment, and we will continue to deliver value to patients around the world by advancing our R&D and resolving challenges.

Global Growth Strategy: Accelerating Value Creation Toward 2035

Establishing a U.S. Business Foundation and Accelerating Business Growth

Viltepso, which we launched in Japan and the U.S. in 2020, is a treatment for DMD that enables the skipping of exon 53 of the dystrophin gene. Since then, Viltepso has built a growing track record of use among patients and has steadily increased our revenue in the U.S., supporting the growth of our business there.
By bringing this product to market, we have established a foundation for sustained business operations in the field of intractable and rare diseases, including market access, insurance reimbursement, patient support programs, logistics and sales systems that ensure a stable supply, and networks with healthcare professionals and patient organizations.
We are leveraging this foundation to accelerate the rollout of our pipeline products.
In the DMD field, in addition to developing multiple exon-skipping drugs, we are also working to develop and in-license therapeutic agents with different mechanisms of action, with the aim of expanding the range of therapies available to underserved patient populations and improving access to care.
These initiatives will enable the Nippon Shinyaku Group to continue delivering sustainable value in the DMD field and fulfill its role as a pharmaceutical company committed to addressing intractable and rare diseases.

Improving Patient Access

In the U.S., ensuring patient access requires a deep understanding of the local healthcare landscape. Led by NS Pharma, our Group is striving to establish a support system designed to deliver meaningful value to each and every patient.
As part of these efforts, our NS Support HUB service for patients and their families provides assistance with insurance reimbursement procedures as well as the support needed to help patients continue treatment. This enables us to facilitate interactions between patients and healthcare providers while supporting the entire process from starting treatment to ongoing care, which helps ensure that patients can access the treatment they need.
These initiatives will enable us to ensure that the value of our products reaches patients while also providing them with sustained access to treatment.



NS Pharma's Initiatives Supporting Our U.S. Business

While the U.S. market tends to favor innovative pharmaceuticals, changes in the business environment, including drug pricing policies, can have a significant impact on profitability, requiring a strategy that takes both opportunities and risks into account.
The Nippon Shinyaku Group has positioned its subsidiary NS Pharma at the core of its U.S. business operations and is working to establish an operating framework that integrates market access, distribution, and patient support. To navigate the unique characteristics of the U.S. healthcare system, we are working to establish a framework that ensures patients have appropriate access to treatment by developing reimbursement and pricing strategies covering both public and private insurance, cooperating with healthcare providers and insurers, and providing patient support programs. We are also coordinating preparations across manufacturing, quality, distribution, and sales to establish a framework for stable product supply and marketing from the initial stages of launch.
These initiatives will enable us to consistently deliver value to patients while driving sustainable business growth.

Employee Voice



Business Strategy Department
Akiyoshi Iwai

Expanding Our Global Business

We are currently working to expand patient access globally by pursuing the rollout of Viltepso in overseas markets while identifying the optimal approach for each country based on its healthcare system and market environment. In addition, Uptravi is already available in 76 countries through our global partnership network. Through these initiatives, we are steadily expanding our capabilities, expertise, and networks to accelerate the global delivery of high-quality, differentiated products.
In China, we have local subsidiaries in Beijing and Tianjin, where we sell our products through sales consignment to local partners while building the foundation for introducing new products to the market.
In Europe, we continue to evaluate the optimal timing for business expansion in light of external factors—including the U.S. Most-Favored-Nation (MFN) framework—and pipeline progress.
Overall, these initiatives will further enhance our ability to deliver value in the field of intractable and rare diseases on a global scale.