LEK™

Japan

On the Cusp of a Cure

Is Asia Pacific Ready for the Precision Era?

On the Cusp of a Cure

Is Asia Pacific Ready for the Precision Era?

Acknowledgements

L.E.K. Consulting would like to acknowledge the contributions of all the Advisory Committee members who have provided critical insights into the development of this report. Specifically, their contributions have guided the assessment of barriers facing the adoption of precision therapies investigated and recommendations to addressing these barriers.

Name	Position	Organisation
Australia		
David Thomas	Chief Scientific Officer	Omico
Jaala Pulford	Chair of the Board	MTPConnect
Christine Cockburn	CEO	Rare Cancers Australia
China		
Baorong Yu	Professor and market access key opinion leader	University of International Business and Economics
Andy Mok	Head of China	Guardant Health
Kevin Huang	Founder and president	China Organization for Rare Disorders (CORD)
Caicun Zhou	Professor and lung cancer key opinion leader	School of Medicine, Tongji University
Japan		
Teruyuki Katori	Representative Director Specially Appointed Professor	Future Institute Wolong, General Incorporated Association Graduate School of Social Sciences, University of Hyogo
Daisuke Sato	Professor	Fujita Health university
Manabu Muto	Professor	Kyoto University Graduate School of Medicine
Masahiro Miyake	Professor	Kyoto University Hospital
Naomi Sakurai	Founder and CEO	Cancer Solutions
Keisuke Shimizu	Leader	Lung cancer HER2 "HER HER"
South Korea		
Paul Lee	Former GM	Gilead Sciences Korea
Jin-Ah Kim	Advocate for rare diseases	Seoul National University Hospital, Department of Genomic Medicine, Rare Disease Centre
Prof Jeonghoon Ahn	Professor of Health Convergence	Ewha University

This report was sponsored by Johnson & Johnson. The research and writing of this report was conducted independently and reflects the unbiased analysis and conclusions of the authors.

Disclaimer

Contents

Executive summary 4

1.0 Introduction 11

1.1 Evolution of medicines 12

1.2 Drivers of growth in the precision era 14

1.3 Definitions 16

1.4 Report development approach 21

2.0 Japan 23

2.1 Introduction 24

2.2 Benefits of the precision therapies 25

2.3 Barriers to access 32

2.4 Recommendations 37

3.0 Appendix 41

References 49

Executive summary

Over the next decade, the nature of disease treatments and the way medical therapies are delivered to patients will dramatically change as a result of technological breakthroughs in diagnostics, big data and artificial intelligence (AI). Cutting-edge scientific advances like genomic profiling are transforming our understanding of what causes disease and are enabling researchers to predict treatment suitability and outcomes more accurately for patients.

Cutting-edge scientific advances like genomic profiling are transforming our understanding of what causes disease and are enabling researchers to predict treatment suitability and outcomes more accurately for patients.

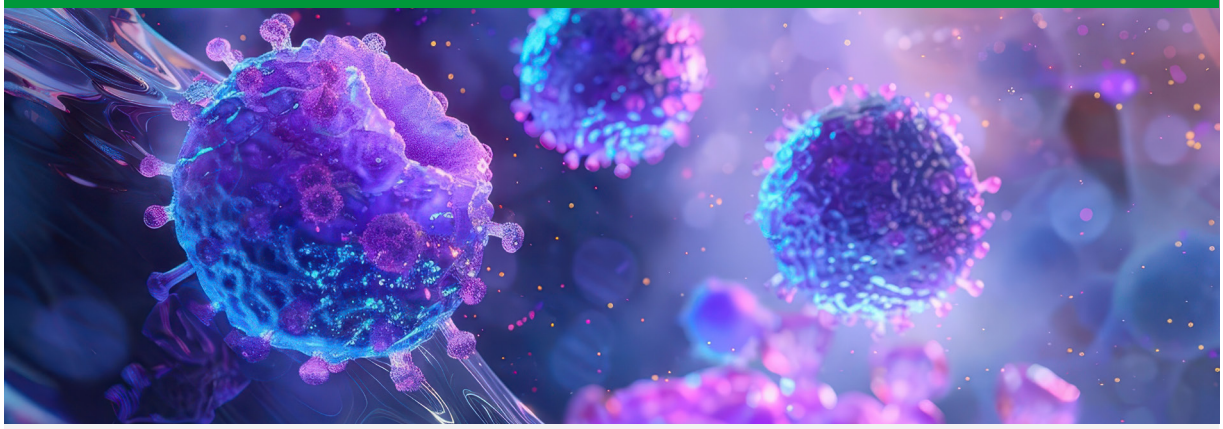
As a result of these advances, we are entering a transformative period in medicine — the 'Precision Era', where the fundamental way in which patients are treated is being redefined. In the Precision Era, medicines will move away from being generically prescribed and administered, small molecule treatments aimed at alleviating symptoms, which require regular medication to manage chronic conditions. Instead, treatments are becoming highly tailored to individual patients and provide patients with the potential for a curative solution, specific to their disease. We call this new wave of innovative disease treatments 'precision therapies'.

Precision therapies are poised for rapid growth over the next decade, driven by technological advancements. They are no longer a distant concept, but are already available for patients in many markets, with a significant pipeline of new precision therapies expected to launch within the next five years. In fact, looking at cell and gene therapies alone, there are nearly 4,000[1] therapies in development. Precision therapies are broader than just cell and gene therapies.

In this report, we include the following technologies as a representative sample of precision therapies:

- **Cell therapies** involve the addition or transplantation of modified human cells into a patient to treat a disease
- **Gene therapies** treat or prevent disease by correcting disease causing DNA mutations in patients
- **Targeted antibody therapies**, which allow the targeted treatment of a broad variety of diseases ranging from cancer to autoimmune conditions, often used in conjunction with innovative diagnostics such as comprehensive genomic profiling
- **Drug-device therapies**, which improve on traditional treatment and drug delivery because the drug is sent directly to tumorous cells, reducing dosage, side effects and damage to healthy cells, and
- **Diagnostic technologies** such as comprehensive genomic profiling which are used to identify specific patient populations for treatment by gene and targeted antibody therapies.

Challenges of access to precision therapies from a patient's perspective



Currently, precision therapies in cancer brings hope to many of us patients. By discovering specific genetic abnormalities, we are provided with the opportunity to participate in cancer genome medicine and clinical trials at an early stage, potentially saving lives like mine. However, not all patients can benefit from this, and in my patient group that focuses on specific genes, we still witness people passing away. While precision therapies holds a beacon of hope, for those who cannot access it, it also casts shadows of despair and anxiety.

In Japan, there are several challenges: the timing of genetic panel testing for cancer is delayed, and even when genetic abnormalities are identified, companion diagnostics are approved based on specific organs. Additionally, there is a lack of compatibility among companion diagnostics, which means that the treatment options can be limited depending on which diagnostic test was used. Furthermore, not all patients receive information about treatments or clinical trials based on genetic test results. It is a significant issue that some patients are unable to progress with their treatment despite the potential for life-saving therapies.

Given this situation, I strongly hope for the development of organ-agnostic genome medicine based on genetics, specifically concerning companion diagnostics. This includes the implementation of genetic panel tests at earlier stages and further advancements in whole-genome analysis. I believe that these steps will alleviate some of the anxiety and concerns of patients and lead to better healthcare that contributes to the lives and health of the entire population.

It is also crucial for us patients to collaborate with many stakeholders to strive for a future where everyone has access to the right treatment. The advancement of precision therapies is a significant step towards saving and protecting all lives, and I look forward to a future with better healthcare for patients.

- Keisuke Shimizu, Leader of Lung cancer HER2 "HER HER"

Each of these technologies is considered part of the Precision Era because they provide enhanced health benefits to patients through a more tailored approach, represent a paradigm shift to how care has been provided previously, and are the focus of significant research and development (R&D) efforts and investment with a wave of new therapies expected to launch in the next five to ten years. Through the rapid developments in these precision therapies, some experts have suggested that we may be on the "cusp of a cure" for diseases that have previously been considered incurable, such as many cancers and genetic disorders.

Precision therapies hold enormous benefits for not just patients, but also their families, carers, healthcare systems and the broader economy. This report focuses on first-of-its-kind research outlining these wide-ranging benefits in detail. However, as with many innovations, capturing the full value of

[1] Australian Government Department of Health and Aged Care, 2024. Minister for Health and Aged Care press conference – 28 August 2024. [online] Available at: https://www.health.gov.au/ministers/the-hon-mark-butler-mp/media/minister-for-health-and-aged-care-press-conference-28-august-2024

precision therapies will require coordinated changes across the broader healthcare ecosystem, including funding models, infrastructure, physician capabilities, patient care and journeys. This report also identifies the key barriers currently limiting adoption and impact and provides actionable recommendations to address them.

Our research and modelling included data from more than 1,000 clinical trials across the key technologies considered. The analysis shows that in addition to patient benefits, these medicines will also deliver a broad range of social and economic benefits. They are projected to reduce healthcare costs, strengthen R&D activity, improve healthcare infrastructure, and training for healthcare providers — all of which will in turn generate greater investment into the sector and productivity improvements. The broader economy will also benefit, with industry driving innovation and creating skilled jobs.

The analysis shows that in addition to patient benefits, these medicines will also deliver a broad range of social and economic benefits.

In addition to direct benefits, improving access to precision therapies will also drive multiplier effects indirectly. Multiplier effects describe how an initial injection of investment into an economy can lead to a larger increase in economic output and employment, as the initial impact ripples through different sectors and stimulates additional economic activity.

However, there are several barriers that restrict patient access to these innovative treatments and prevent the realisation of the multitude of benefits described above. Recognising the potential benefits of precision therapies, L.E.K. Consulting has taken a systematic approach to assessing the barriers to adoption and solutions that can address these barriers.[2]

Through in depth consultation with independent experts in patient advocacy, industry, government and economic policy, we have assessed four key Asia Pacific (APAC) markets — Australia, China, Japan and South Korea. For Japan, the specific findings of the studies can be seen on the next page.

As illustrated in the exhibit below, precision therapies offer enormous health and economic benefits to all Japanese citizens and to the Japanese economy. Realising these benefits will require a coordinated effort across all stakeholders involved – industry, government, clinicians and the broader healthcare workforce, researchers and investors.

If such coordinated action is not taken, Japan risks ever-increasing healthcare expenditure that compromises social equity and justice and delivers sub-optimal health outcomes for Japanese citizens.

Japan

Benefits (10-year cumulative view)

1.5Mn patients
Improved treatment & patient outcomes for over 1.5 million patients including improved access to precision therapies, supporting diagnostics and clinical trials

Over ¥860Bn JPY
in avoided healthcare costs through curative treatments for previously untreatable diseases

¥17Tn JPY
invested across R&D, diagnostics and manufacturing supporting development of new skills and expertise

Potential of ¥100Tn JPY of investment
in economic activity and 250,000 jobs through multiplier effects of investment and patient benefits across the broader community

Japan

Solutions

Enhance the regulatory approval systems to better evaluate precision therapies:

- Align regulations and insurance systems and tailored value assessment methods
- Involve appropriate experts in evaluations

Ease insurance coverage regulations for combination diagnostics (CDx)/comprehensive genomic profiling (CGP)

- Involve appropriate experts in evaluations of CDx
- Ease CGP regulations and insurance coverage

Expand access to innovative therapies and compassionate use programs

- Enhance access to treatment outside of insurance coverage
- Enhance access to compassionate use programmes

Enhance research infrastructure and collaboration for precision therapies innovation

- Facilitate partnerships that encourage innovation across sectors and borders
- Create a more supportive R&D environment by reducing administrative burden and improving compensation for researchers
- Improve clinical trial recruitment efficiency and transparency
- Establish medical and genomic data infrastructure for use in clinical research

[2] Jackson, Stuart and Trakhtenberg, Ilya, 2025, Predictable Winners, Stanford University Press

Regional perspectives

At the regional level, there is huge potential for economic investment in R&D, diagnostics and manufacturing as well as healthcare cost savings over the next 10 years. Importantly, millions of patients will benefit.

This first-of-its-kind research evaluates the whole system benefits of precision therapies in Asia Pacific, with a focus on Australia, China, Japan and Korea:

- Economic investment of around **$450Bn USD** over the next 10 years across the four markets
- Creation of **almost 1.2Mn** jobs
- Additional indirect economic growth through a multiplier effect on adjacent industries as the initial growth ripples through more sectors and stimulates economic activity culminating to **an estimated $1.615Tn USD growth**
- Greater workforce participation from over **24Mn patients and caregivers**

As conservative projections of future patient numbers and treatment paradigms were used, the actual impact is likely to be much higher than forecasted in this report as further treatments are brought to market and innovative treatments are likely to be offered earlier in the patient pathway.

There is huge potential for economic investment in R&D, diagnostics and manufacturing as well as healthcare cost savings over the next ten years.

Projected benefits of precision therapies
(cumulative over 10 years)

	Australia	China	Japan	South Korea
Improved outcomes	**250k** patients	**10.5Mn** patients	**1.5Mn** patients	**400k** patients
Invested in R&D, Diagnostics and Manufacturing	Over **$50Bn** AUD $32Bn USD	Over **¥1.45Tn** RMB $200Bn USD	Over **¥17Tn** JPY $110Bn USD	Over **₩61Tn** KRW $44Bn USD
Avoided healthcare costs	**$2.6Bn** AUD $1.7Bn USD	**¥216Bn** RMB $30Bn USD	**¥860Bn** JPY $5.7Bn USD	**₩2.2Tn** KRW $1.6Bn USD

Each of the four markets assessed in this report are not alone in facing challenges to enabling access to precision therapies. In fact, there are three common barriers that we have observed across the four APAC markets investigated as described below.

	Common barriers	Common solutions
1	**The first was the lack of a clear, fit-for-purpose regulatory and reimbursement pathways to properly evaluate these paradigm-shifting precision therapies** The impact of this being delayed access to therapies by patients and lower reimbursement for diagnostics and novel therapies, resulting in higher out-of-pocket costs and affordability constraints	**Develop streamlined, fit-for-purpose regulatory and reimbursement pathways for the precision therapies that recognise wider value of these medicines to patients, their carers and families, and the broader society and economy** Including more timely access to diagnostics, curative therapies for patients, improved affordability either through pricing, reimbursement or insurance programs for both treatments and diagnostics
2	**Low patient and healthcare provider awareness of these novel therapies and the benefits they offer to patients, their families and friends and society-at-large** The impact of this being potential misdiagnosis and delays, reluctance of treating physicians to prescribe patients novel therapies and difficulty in access to and navigation of the environment by patients	**Increase efforts to educate healthcare providers, patients and the broader public about precision therapies** Supporting healthcare providers with clinical guidelines and medical information on these innovative therapies and assisting them and advocacy groups in navigating the complex patient pathways
3	**Key healthcare infrastructure and investment to support patient access to these medicines** Such as improving access and number of clinical trials that local patients can participate in, access to information about novel therapies, access to molecular profiling (which includes both genomic and protein-based components) as the standard of care, leveraging genomic data for R&D and to build knowledge, and supporting innovation and R&D to continue progress of development in precision therapies	**Encourage cross-sectoral collaboration between the public sector and healthcare industry to foster innovation and promote access** Supporting industry to continue its investment into new therapies and diagnostic tools locally, to improve in-country accessibility and the development of local resources and talent

The recommendations in this report need to be implemented with urgency if these four markets (Australia, China, Japan and South Korea) are to gain significantly from the multitude of benefits that precision therapies hold. Governments, the healthcare industry, its professionals and patient advocacy groups must all work together collaboratively to ensure the full potential of the precision therapies is realised.

1.0 Introduction

1.1 Evolution of medicines

For much of the 20th century, medical innovation was focused on managing symptoms. While discoveries like penicillin were lifesaving, most innovations only extended life rather than provide cures. Over the last 30 to 40 years, medical advancements have accelerated, driven by advances in technology and our understanding of the human genome.

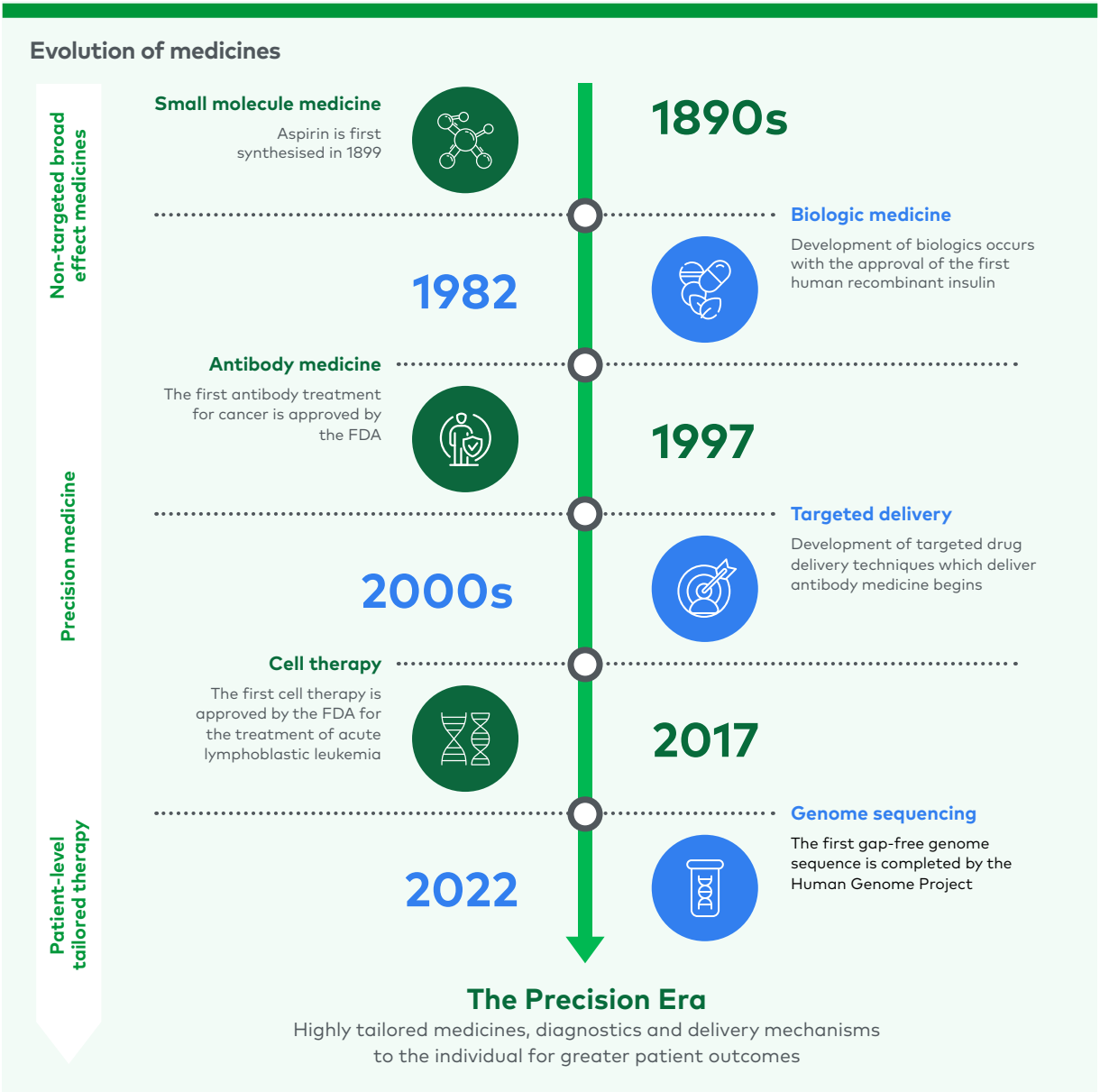
In the late 20th century, biologics — natural molecules in humans and animals — were developed to address disease symptoms more effectively.

At the turn of the century, immunotherapies, such as antibody therapies, emerged. These treatments harness the body's immune system to fight diseases.

In the 21st century, targeted drug delivery techniques improved the effectiveness of antibody therapies, reducing side effects and offering patients more freedom from regular treatments.

Technological advancements over the last five years, including tools that identify and edit disease-causing mutations and the achievement of the first complete genome sequence, have marked a turning point in the acceleration of medicine and healthcare.

These breakthroughs are causing a revolution in the way patients are treated. In contrast to



Technological advancements over the last five years have marked a turning point in the acceleration of medicine and healthcare.

traditional treatments, novel cancer treatments are becoming more personalised through individual genomic information and data, and offering the potential for a curative solution specific to a disease for diseases previously thought to be incurable. This enables clinicians to match patients with the most effective treatments, ushering in the Precision Era.[3]

In the context of an increasing burden of disease, this innovation is critical. Precision therapies are becoming the gold standard in personalised treatment and to date, have largely been aimed at treating cancer — one of the leading causes of death in APAC and globally.[4] In 2024, over 20Mn people globally will be diagnosed with cancer and over 10Mn will die of the disease — reflecting a sharp and continuing rise in prevalence.[5]

Looking forward, treatments may no longer require ongoing medication or regular doses. Instead, highly individualised cell and gene therapies may be one-time treatments that can slow or stop disease progression — and even cure or prevent disease.

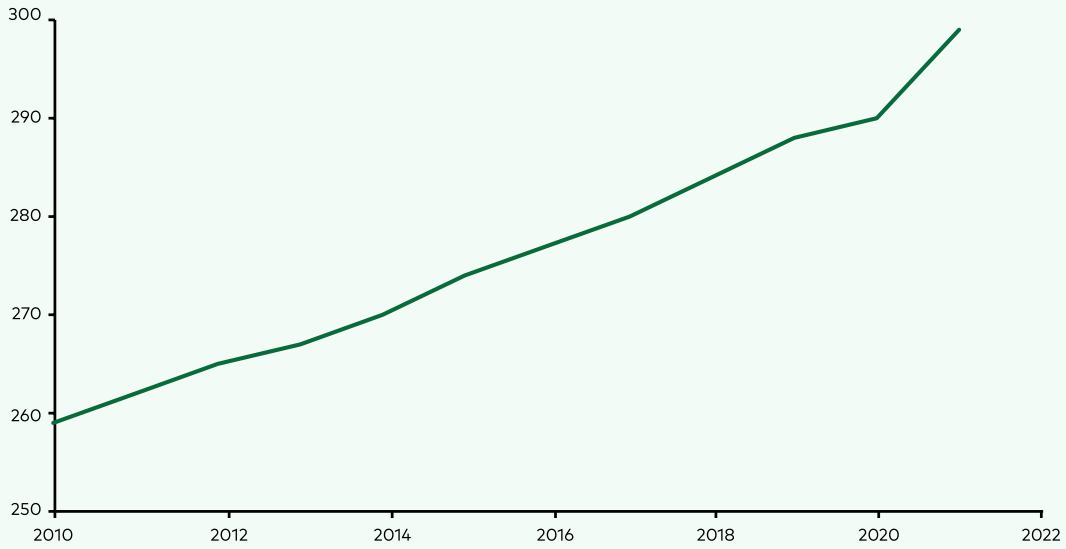
Over the next five to ten years, the pharmaceutical and biotechnology industries are expected to deliver a surge of precision therapies, especially in oncology, immunology, and neurology. In cell and gene therapies alone, nearly 4,000 therapies are currently being developed around the world.[6]

Beyond cancer, precision therapies can be used for rare, often poorly funded, genetic diseases. Though an individual rare disease may affect fewer than five in every 10,000 people, they collectively impact around 8% of the global population.[7] These diseases are often progressive and incurable, affecting patients' quality of life and life expectancy.

Precision therapies offer hope by significantly advancing the standard and options of care for these previously untreatable conditions.

Looking forward, treatments may no longer require ongoing medication or regular doses. Instead, highly individualised cell and gene therapies may be one-time treatments that can slow or stop disease progression — and even cure or prevent disease.

Global incidence of cancer (2011-21)
Cases per 100,000 people

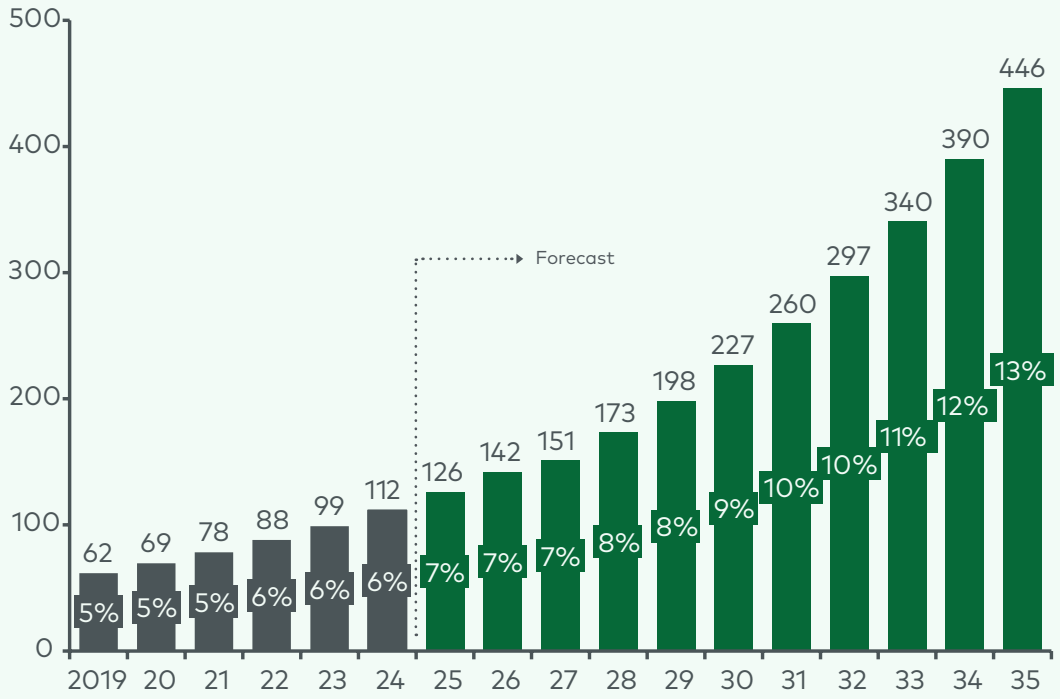


[3] National Institutes of Health (NIH), 2022. Researchers generate the first complete, gapless sequence of a human genome. [online] Available at: https://www.nih.gov/news-events/news-releases/researchers-generate-first-complete-gapless-sequence-human-genome
[4] Institute for Health Metrics and Evaluation (IHME), 2024. Global Burden of Disease (GBD) Data. [online] Available at: https://www.healthdata.org/research-analysis/gbd-data
[5] Institute for Health Metrics and Evaluation (IHME), 2024. Global Burden of Disease (GBD) Results. [online] Available at: https://vizhub.healthdata.org/gbd-results/
[6] Australian Government Department of Health and Aged Care, 2024. Minister for Health and Aged Care press conference – 28 August 2024. [online] Available at: https://www.health.gov.au/ministers/the-hon-mark-butler-mp/media/minister-for-health-and-aged-care-press-conference-28-august-2024
[7] European Commission, 2024. Rare diseases. [online] Available at: https://health.ec.europa.eu/rare-diseases-and-european-reference-networks/rare-diseases_en

1.2 Drivers of growth in the precision era

Precision therapies are expected to grow at 14.5% annually to reach $446Bn USD globally by 2035, outpacing broader medicine market growth of 6.6%.[8, 9]

Indicative global market size for precision therapies (2019-35)
$Bn of USD (percentage of pharmaceutical market)



[8] Statista, 2024. Global spending on precision medicine treatments from 2020 to 2027. [online] Available at: https://www.statista.com/statistics/1420946/spending-on-precision-medicine-treatments-globally/
[9] IQVIA, 2024. The global use of medicines 2024: Outlook to 2028. [online] Available at: https://www.iqvia.com/insights/the-iqvia-institute/reports-and-publications/reports/the-global-use-of-medicines-2024-outlook-to-2028

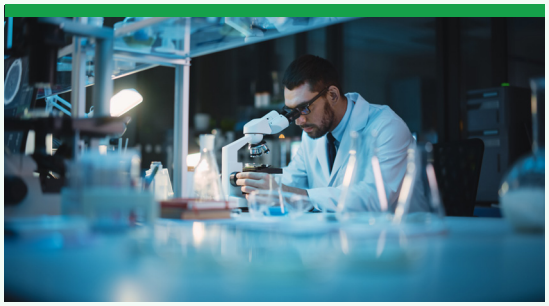
Several factors are driving the rapid growth of precision therapies

Medical unmet need

- Numerous orphan diseases remain with identified genetic causes but have complex or unclear pathophysiology
- Current standards of care in oncology using chemotherapy exhibit low response rates

Biomedical research

- Increasing R&D investment into biomarker discovery and development of precision therapies, driven by the promise of improved patient outcomes



Diagnostic advancements

- Advanced diagnostic techniques (e.g., next-generation sequencing, or NGS) facilitating biomarker-based testing
- Expanding precision diagnostic use cases (e.g., molecular testing in oncology evolving from limited late-stage therapy guidance to full integration from screening to post-treatment surveillance)

Big data and AI

- Availability of big data to inform medical discoveries and predict disease and treatment outcomes

Policy evolution

- Growing recognition of the need for improved test access to harness the benefits of precision therapies
- Expanding guideline inclusion enabling access to precision therapies for an increasing number of indications

To keep pace with these advancements, healthcare systems will need to adapt to provide patients with access to these treatments.

- **Treating clinicians** will require training on when and how to prescribe these therapies, and nurses and other healthcare workers will need to learn how to support patients undergoing treatment.
- **Regulatory bodies** must develop new methods to assess the safety and efficacy of these treatments, as traditional models may not apply.
- **Policy bodies** will also need to collaborate with industry to create new reimbursement models that ensure patients can afford these life-changing treatments.

1.3 Definitions

Precision therapies can have several interpretations based on what therapies are included in scope. In its broadest definition, it can include first-generation biologic medicines that require a simple, single biomarker test to determine suitability for a given patient. These have been available for over the last decade.

This report focuses on a narrower set of therapies that are representative of precision therapies. These therapies are designed to deliver more tailored diagnosis and treatment — therapies that some experts believe are on the cusp of a cure in terms of their potential to treat and cure patients.

Precision therapies are designed to deliver more tailored diagnosis and treatment — therapies that some experts believe are on the cusp of a cure in terms of their potential to treat and cure patients.

Four novel technology types have been assessed for the purposes of this report — cell therapy, gene therapy, targeted antibody therapy, and drug device combination therapies as outlined below. Companion diagnostics can be used in conjunction with these technologies and therapy types to identify more accurately those patients likely to benefit from treatment.

Technology types assessed for the purposes of this report

Cell therapy

Cell therapies that involve the genetic modification of a patient's own cells, utilising the regenerative and immune-activating properties of cells to treat disease

Gene therapy

Gene therapies treat or prevent disease by correcting disease-causing DNA mutations in patients

Targeted antibody

Antibody therapies can target specific cells and utilise the body's immune response or deliver disease fighting agents to cause cancer cell death. When combined with genomic profiling, those patients who benefit materially can be identified based on their biomarkers, avoiding unnecessary treatment of patients who will not benefit

Drug device combination therapy

Drug device combination therapies offer an improved delivery mechanism for existing therapies for a range of diseases, using novel devices or techniques which deliver the therapy to the location of disease

Companion diagnostics

Precision therapies can also be extended to diagnostic tools such as comprehensive genomic profiling (CGP) that are specifically required to identify populations for treatment by gene and antibody biomarker-driven therapies

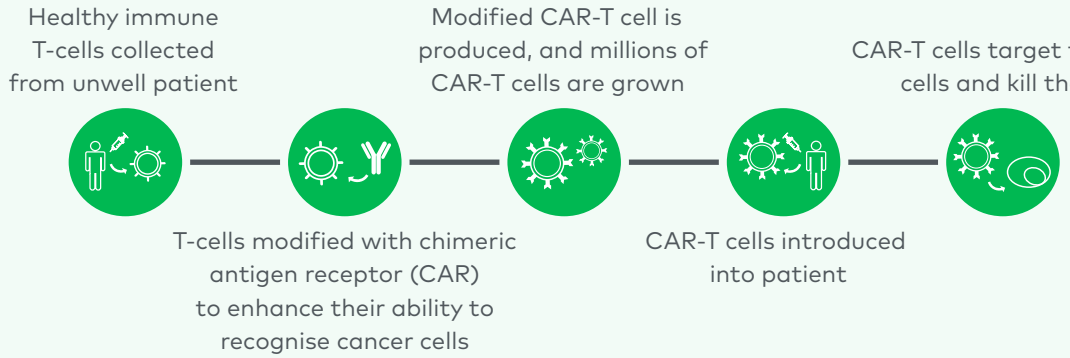
1.3.1 Cell therapy

Cell therapy involves the addition or transplantation of modified human cells into a patient to treat a disease. These cells are generally modified outside of the patient and then re-introduced and may be derived from the patient or separate donor.

The cell therapy market is driven primarily by the development of chimeric antigen receptor T-cell (CAR-T) therapy which uses genetically engineered immune cells from a patient to target and destroy tumour cells, in particular blood cancers, such as multiple myeloma and lymphoma.

An emerging CAR-T technique is non-personalised or "off-the-shelf" therapies which use healthy donor cells instead of the patient's own cells. Approved donor cells are modified and able to treat multiple patients, overcoming the limitations of individual manufacturing of cell therapies for each patient.[11]

CAR-T technology for cancer cell elimination[10]



[10] Sheykhhasan M., et al. Cancer Gene Therapy, 2024. CAR T therapies in multiple myeloma: unleashing the future. [online] Available at: https://www.nature.com/articles/s41417-024-00750-2
[11] Memorial Sloan Kettering Cancer Center, 2024. Off-the-shelf CAR cell therapy for multiple myeloma shows promise. [online] Available at: https://www.mskcc.org/news/shelf-car-cell-therapy-multiple-myeloma-shows-promise

1.3.2 Gene therapy

Most key functions in cells are carried by proteins, which carry out these functions based on the information provided by DNA. "Typos" or errors in our DNA can lead to a partial or complete loss of these cell functions. This is the origin of genetic disease.

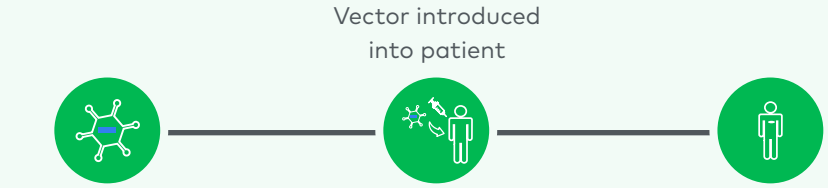
Gene therapy treats or prevents disease by correcting the DNA mutation using vectors, which are usually viruses that function as a 'vehicle' to deliver therapeutic genetic material, such as a working gene, directly into a cell.[12]

Gene therapy involves modifying a patient's genes outside the body by mixing them with a vector before returning them to the patient or by delivering the genes with vectors directly into the patient's body.

Example diseases treated by gene therapies include inherited retinal diseases, spinal muscular atrophy, Duchenne muscular dystrophy and haemophilia.

Ex vivo gene therapy

In vivo gene therapy



[12] American Society of Gene & Cell Therapy (ASGCT), 2024. Vectors 101. [online] Available at: https://patienteducation.asgct.org/gene-therapy-101/vectors-101

1.3.3 Antibody therapy

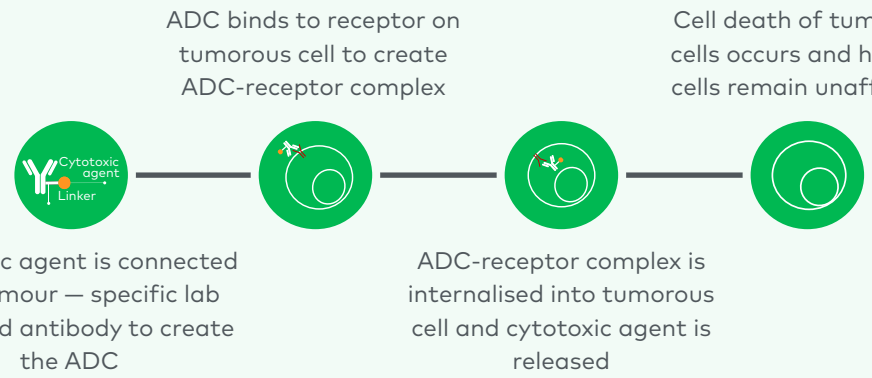
Antibody therapies allow the targeted treatment of a broad variety of diseases ranging from cancer to autoimmune conditions. Antibody therapies are commonly being used to treat a range of solid tumour cancers like lung, colorectal and melanoma.

In cancer treatment, antibody therapy involves the delivery of antibodies which identify and target specific tumour cells. In binding to the tumour cell, the antibody can then 'mark' these cells for destruction by the existing immune system.

Antibody therapies can also release a particular drug upon the binding of an antibody to the targeted receptor, this is called Antibody-Drug Conjugate therapies (ADCs) and works by attaching antibodies to toxic substances. When the antibody binds to a tumour cell, the toxic substance is released and destroys the tumour cell.

More recent advancements in these therapies have seen the development of radioligand therapies, where radioactive isotopes are attached to molecules (called ligands) that specifically bind to tumour cells. Once attached, they deliver targeted radiation directly to the tumour sites.

Antibody-drug conjugate therapy



1.3.4 Drug device combination therapies

Drug device combination therapies are used to treat patients for a range of diseases and conditions. Recent technology advancements for drug devices have seen innovation in how small-molecule drugs, such as chemotherapy is delivered for solid tumour cancers.

These technologies improve on traditional treatment and delivery because the drug is sent directly to tumorous cells, reducing dosage, side effects and damage to healthy cells.

The implantable nature of many of these drug devices also reduces the length and frequency of drug administration.

These therapies are being used to treat a range of solid tumour cancers including bladder, carcinoma, prostate, breast and pancreatic cancer.

Example targeted drug delivery mechanisms

Chemotherapy delivered directly via catheter to bladder

Encapsulated slow-release hormone implant delivered via injection to reduce tumour growth

Wafer containing slow-release chemotherapy surgically inserted into patient

1.3.5 Companion diagnostics

Companion diagnostics are tests that determine whether a particular therapy is suitable for a patient based on their unique genomic profile.

For example, comprehensive genomic sequencing is a type of companion diagnostic that reveals genetic alterations and provides invaluable information to guide treatment options.

Comprehensive genomic profiling

Tumour tissue or blood sample taken from unwell patient

Genetic material from sample is analysed using next-generation sequencing (NGS) technology

The genomic data is interpreted to identify genetic mutations that correlate with specific therapies, which can then be administered to the patient

1.4 Report development approach

In this paper, we outline the wide-ranging benefits of these medical innovations to patients, their carers and families, and the broader community. We also address the current barriers to accessing these treatments by providing recommendations across four key Asia Pacific healthcare markets – Australia, China, Japan, and South Korea that were reviewed as part of the study. These markets have been selected because they possess some of the largest and most established economies and health systems in the region.

The Executive Summary provides a regional overview and identifies the key themes across the four markets in the study. This is followed by an introduction to provide definitions on precision therapies, the technologies and therapies underpinning it and an overview of their mechanisms of action, which are the same across the four markets.

The remainder of the report is structured around the following three categories for Japan:

1. **Benefits** in terms of economic contributions, cost saving, and life years gained by patients
2. **Barriers** that currently impact the access to, and wider uptake of, precision therapies
3. **Recommendations** to progress precision therapies specific to the needs of each country

1.4.1 Benefits framework

We have used a benefits framework (shown in the exhibit below) to assess the qualitative and quantitative benefits of precision therapies. These medicines have been shown to increase life expectancy and offer significant improvements in quality of life for patients. However, the benefits are much broader than patient benefits, and extend to health system benefits, uplift in economic activity, including multiplier effects. The benefits analysis was undertaken by L.E.K. Consulting, leveraging existing case studies and assessments, and a robust set of economic, and clinical data.

The benefits have been modelled conservatively based on the therapies and treatment paradigms available today for patients in precision therapies, leveraging existing clinical data.

The technologies and the applications of these to different disease states used in the benefits modelling is not representative of the entire potential for precision therapies, the examples that have been used represent the largest and most applicable proven patient use cases that exist at the time of writing the report.

The benefits have been modelled conservatively based on the therapies and treatment paradigms available today for patients in precision therapies, leveraging existing clinical data.

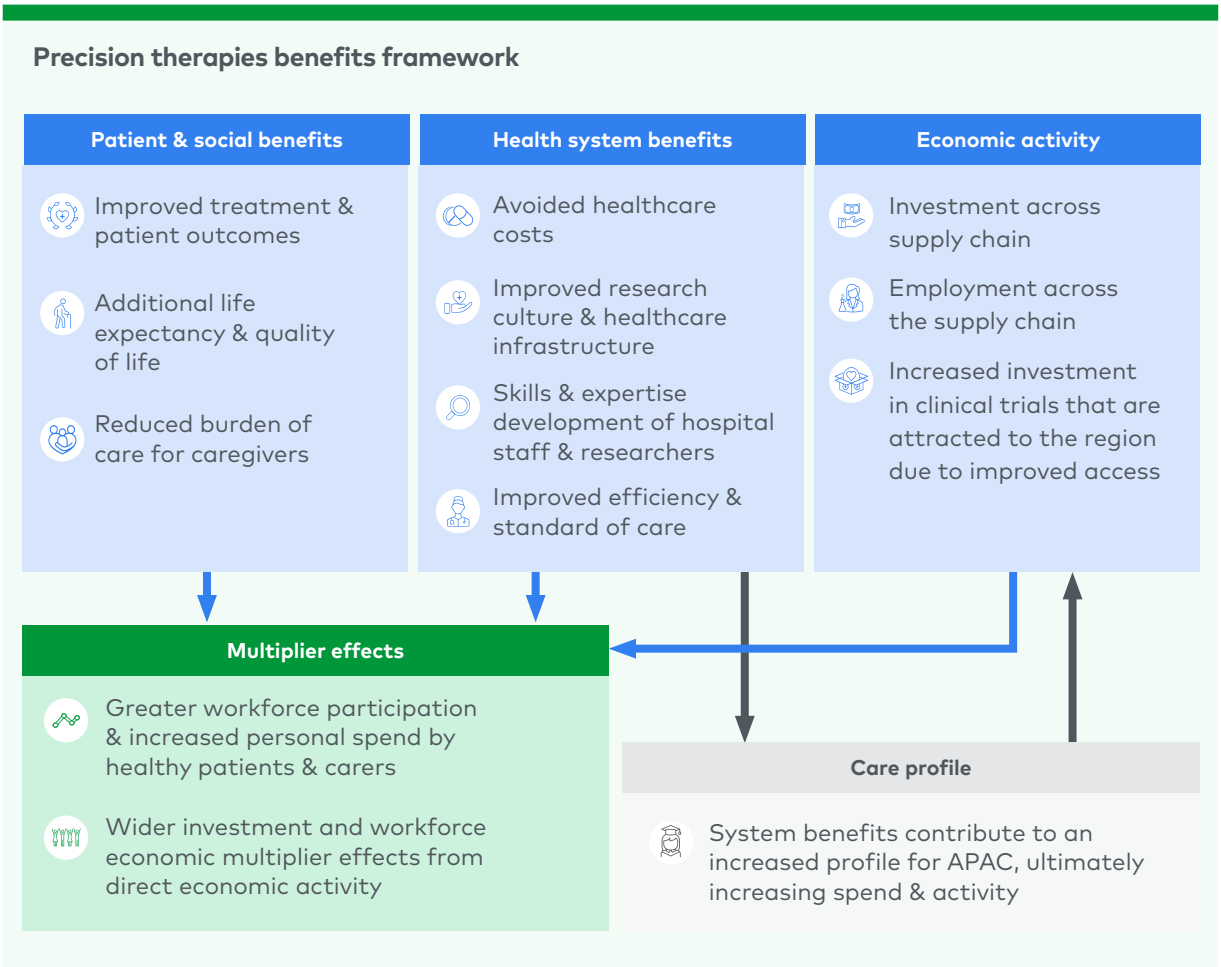
The actual benefits realised may be much greater over time as more such therapies become available earlier to patients in their treatment journey and deliver benefits to not only a larger number of patients, but also deliver even greater benefits to each patient through earlier treatment.

Additional detail on the calculation steps and values are provided in the Appendix.

Patient and social benefits have been determined by reviewing each technology type and selecting the most relevant disease category (e.g. blood cancers for cell therapy) for which a precision therapy exists. Research of published clinical trial and real-world results was then undertaken to determine the reported additional life expectancy compared to the existing standard of care and extrapolating this to the entire patient population that could be eligible for treatment in each country. It is expected this number will significantly increase as more treatments become available to treat other disease categories, and as existing treatments show results in earlier lines of use, e.g. used first line for treatment as opposed to after existing older therapies have been used without success.

Health system benefits take the improved life expectancy and what the cost would have been for each additional year of treatment under the standard of care and calculates the implied cost saving (or 'avoided healthcare cost'). Costs for standard of care are based on published data sets for each country.

Economic activity are the financial investment and creation of new employment in the pharmaceutical, medical devices and diagnostic



supply chain to support the development of local infrastructure required for precision therapies, by local and foreign agencies. The quantification of the benefits is based on the observed investment and employment in the pharmaceutical sector from published datasets, adjusted for the proportion of precision therapies that are expected over the next ten years.

Multiplier effects are calculated based on two aspects:

- The recovered population's contribution back into society and the workplace — increased workforce participation and personal spend by healthy patients and their carers
- The broader ranging flow on benefits that are created from the "economic activity benefits" in the broader ecosystem — such as taxes paid on new jobs created to support precision therapies
- Secondary sources have been used to substantiate this opportunity size

In developing the analysis to determine the market potential, existing clinical trial data was used:

- The selection of clinical trials was based on the availability of data for the precision therapies aligned to the therapy areas outlined in the introduction (biomarker based therapy/gene/cell therapy)
- In total, this covered approx. 900-1,100 clinical trials across the 4 countries (Australia: 130-190, China: 500-620, Japan: 120-160, South Korea: 110-150 (noting there is some variation year to year)
- Selection of the included therapies was based on FDA approvals and their availability in each region

1.4.2 Barriers and recommendations

The barriers and recommendations were developed with the input of independent advisory committees established for each country. These advisory committees were made up of key opinion leaders and healthcare professionals, patients, and patient advocacy group representatives, current and former industry representatives and in some countries, previous government ministers. A series of sessions were run in each country over a two to three month period to gather information and distil this into the key messages.

We have taken this approach to ensure that the paper reflects the current requirements specific to each country and is actionable by government and industry groups.

2.0 Japan

2.1 Introduction

Japan's National Health Insurance system ensures that all citizens have access to high-quality healthcare, which has contributed to Japan having one of the highest average life expectancies in the world.

The government has taken important steps to support the development of precision therapy, with institutions like the National Cancer Center Hospital leading in genomic-based cancer treatment.[13] However, Japan is now facing new challenges, including rising healthcare costs due to its ageing population and the growing use of advanced medical technology. Additionally, the declining working-age population, linked to low birth rates, places further strain on the sustainability of the National Health Insurance system.

The government has taken important steps to support the development of precision therapy, with institutions like the National Cancer Center Hospital leading in genomic-based cancer treatment.

To maintain the National Health Insurance system and secure its future, it is important to manage the growth of healthcare costs. One of the measures implemented by the Japanese government, together with the Ministry of Health, Labour and Welfare, is the biennial revision of medical service fees (*shinryo houshu*), which covers medical, dental, dispensing, and drug prices. Among these, drug prices have seen the most significant reductions, through both regular and special price reductions.

While these price reductions aim to control costs, there are concerns that they may also reduce Japan's attractiveness in the global pharmaceutical market. As a result, there is growing apprehension that new medicines and medical technologies are not being introduced as quickly in Japan. This trend, often referred to as "drug loss" or "drug lag" risks limiting access to innovative treatments for Japanese citizens.

Given this backdrop, a key issue is to balance the promotion of innovation — whether developed domestically or imported-and controlling rising healthcare costs to sustain the National Health Insurance system.

For example, certain gastrointestinal stromal tumour (GIST) treatments like avapritinib and ripretinib which are already approved by the U.S FDA have shown highly effective results but there are currently no plans to bring these drugs to market in Japan, despite strong demand from patients.[14]

Given this backdrop, a key issue is to balance the promotion of innovation — whether developed domestically or imported — and controlling rising healthcare costs to sustain the National Health Insurance system. The Basic Policy on Economic and Fiscal Management and Reform 2024 (Honebuto no Hōshin) also highlights the importance of reviewing prices in 2025 to support innovation[15], ensure a stable supply of medical treatments, and consider the impact of rising prices on the National Health Insurance system.

[13] Shimada, K., Nakamura, K. and Yamamoto, N., 2023. Mission of the National Cancer Center Hospital in Japan to promote clinical trials for precision medicine. Cancer Biology & Medicine, 21(1), pp. 1–3. Available at: https://doi.org/10.20892/j.issn.2095-3941.2022.0643
[14] A New Drug Lag: Increasing Number of Unapproved Drugs with No Plans for Development . [online] Available at: https://medical.nikkeibp.co.jp/leaf/all/series/cancernavi/202210/576994.html
[15] 'Regarding the Basic Policy on Economic and Fiscal Management and Reform 2024,' the 'Grand Design and Action Plan for a New Form of Capitalism 2024 Revised Edition,' and the 'Regulatory Reform Implementation Plan,' among others. [pdf] Available at: https://www.mhlw.go.jp/content/12401000/001270729.pdf

2.2 Benefits of precision therapies

Expanding access to precision therapies offers wide-ranging benefits across multiple areas, including healthcare, the economy and society. These benefits are expected to have positive effects on the well-being of the Japanese population and contribute to long-term improvements in healthcare delivery.

The benefits proposed below cover a range of factors that will impact patients, their communities, and the broader pharmaceutical, diagnostics and healthcare industries.

Patient and social benefits cover the improved quality of life of patients and their carers as a result of receiving precision therapies.

Benefits to the healthcare systems deliver cost savings and increased capability in the system. Finally, the economic benefits and multiplier effects are in the form of investment dollars and additional value generation from increased direct investment in infrastructure and jobs across the pharmaceutical and diagnostic value chains.

Importantly, the benefits outlined and quantified below, relate only to the latest generation of precision therapies, i.e. the four technology areas which have been the focus of this report. The benefits, if applied to a broader definition of precision therapies, would be multiples larger.

Cumulative benefits of precision therapies
(2025-35)

Patient & social benefits

- Improved **treatment & patient outcomes** for **c.1.5M addressable patients**
- **c.1.3M cumulative years of life gained** across the addressable patient pool
- **Reduced burden of care for 1.4-1.5M caregivers** as a result of patients treated

Health system benefits

- **c.¥863Bn JPY ($5.7Bn USD) in avoided healthcare costs** from treatment of addressable patient pool
- Improved **research culture & infrastructure** in healthcare
- Hospital staff and researchers **develop new skills and expertise**
- Improved **standard of care and higher efficiency**

Economic activity

- **c.¥17Tn JPY ($1.1Bn USD) invested** across the **pharmaceutical value chain** as well as **c. ¥2.4Tn JPY ($15Bn USD)** in the **diagnostics value chain**
- Supporting up to **c.41,000 highly skilled jobs** in the **pharmaceutical value chain** with additional jobs in the **diagnostics industry and health system**
- **Clinical trial activity generating ¥123-176Bn JPY** ($0.8-1.1Bn USD), providing patients with often **free treatment access** and **upskilling the healthcare workforce**

Multiplier effects

- Greater **workforce participation, increased personal spend** by **c.1.5M healthy patients & 1.4-1.5M caregivers**
- **Economic multiplier effects** from economic activity of 1-6x generating **¥17-102Tn JPY ($112-670Bn USD)** and supporting **41,000-247,000 jobs**

2.2.1 Patient & social benefits

Precision therapies have the potential to improve health outcomes for at least 1.5Mn Japanese people between 2025 and 2035.

Precision therapies have the potential to improve health outcomes for at least 1.5Mn Japanese people between 2025 and 2035.

This progress can be attributed to several factors: (1) more effective treatments that provide longer-lasting results, (2) the introduction of therapies for diseases that were previously untreatable, and (3) improved safety through more targeted, personalised treatments.

Over the next ten years, these medicines are projected to add approximately 1.3Mn years of life for the eligible patient population.

Further benefits are expected as existing therapies are expanded and used earlier in patients' treatment journeys. Currently, many of these therapies are administered as third or fourth-line treatments, as they have not yet been widely tested as early stage interventions. As more data is collected to demonstrate the efficacy of precision therapies in earlier stages, the life year gains per patient are expected to increase.[16] Additionally, as innovation continues and new therapies are developed for a wider range of diseases, more patients will have access to these life-changing treatments.[17]

For example, CAR-T therapies are currently used for patients with refractory or relapsed blood cancer who have already undergone at least three prior treatments. However, studies have shown that using CAR-T therapy earlier in the treatment process results in better patient survival rates.[18]

By making these therapies available earlier, a larger number of patients could benefit from these advanced treatments.

Additional patient life years

	Incident addressable patients (2025-35)	Additional life years per patient with precision therapies compared to SoC	Total additional life years from precision therapies (2025-35)
Cell therapy	**67,874** blood cancer patients	**3.37** life years	**228,856** life years across population
Gene therapy	**2,191** rare genetic disease patients	**6.71** life years	**14,706** life years across population
Targeted antibody & drug device combination therapy	**1.452M** solid tumour cancer patients	**0.719** life years	**1M** life years across population

[16] Cappell, K.M. and Kochenderfer, J.N., 2023. Long-term outcomes following CAR T cell therapy: what we know so far. Nature Reviews Clinical Oncology, 20, pp. 359–371. Available at: https://www.nature.com/articles/s41571-023-00754-1
[17] AstraZeneca, 2024. Precision Medicine. [online] Available at: https://www.astrazeneca.com/r-d/precision-medicine.html
[18] OncLive, 2024. Quadruplet therapy and early-line CAR T-cell therapy approvals mark shifting standards in multiple myeloma. OncLive. [online] Available at: https://www.onclive.com/view/quadruplet-therapy-and-early-line-car-t-cell-therapy-approvals-mark-shifting-standards-in-multiple-myeloma

Precision therapies bring significant social benefits, not only for patients, but caregivers and the broader community.

By improving treatment effectiveness, precision therapies can ease the burden on caregivers reducing the time they spend caring for loved ones and relieving both the emotional and financial stress associated with serious illnesses like cancer. Given that over 90% of patients treated with precision therapies have caregivers, this could reduce caregiving responsibilities for approximately 1.4 to 1.5Mn people in Japan.[19]

Given that over 90% of patients treated with precision therapies have caregivers, this could reduce caregiving responsibilities for approximately 1.4 to 1.5Mn people in Japan.

2.2.2 Health system benefits

Precision therapies can deliver four key benefits to the healthcare system as described in the following section. They can reduce the cost of healthcare, improve the culture of R&D, upskill the healthcare workforce and generate improvements in standard of care.

2.2.2.1 Avoided healthcare cost

Precision therapies could lead to substantial savings for Japan's healthcare system. From 2025 to 2035, it is estimated that treating patients with more effective therapies could save approximately ¥863Bn JPY ($5.7Bn USD), based on a projected patient population of 1.5Mn. These savings come from fewer hospital admissions, reduced medication costs and fewer specialist appointments, as patients remain healthier for longer periods.[20]

2.2.2.2 Improved research culture and healthcare infrastructure

Investments in precision therapies are driving the development of cutting-edge research infrastructure in Japan. Facilities like the Centre for Cancer Genomics and Advanced Therapeutics and biobanks, such as the Tohoku Medical Megabank Project and Biobank Japan, play a key role in advancing research and translating scientific findings into clinical practice.

- Centre for Cancer Genomics and Advanced Therapeutics (C-CAT): Launched in 2018, the centre serves as a platform for genomic analysis and data sharing, helping integrate genomic data into clinical practice. It works closely with an Expert Panel of healthcare professionals to ensure patients receive tailored care based on their genetic profiles.[21]
- Tohoku Medical Megabank Project and Biobank Japan: Established in 2011, this biobank collects biological samples and health data from local residents. It supports genomic research by providing a valuable resource for studying genetic risk factors and developing personalised treatments. Similarly, Biobank Japan has accumulated a vast repository of genetic and clinical data.[22]

Precision therapies impact on patients' families and caregivers

Fewer side effects, shorter recovery periods following treatment and lower risk of disease recurrence

Ability to experience life milestones and participate in the community

Enhanced physical, cognitive, emotional and social functioning

Relief from the psychological burden of living with an incurable or terminal disease

[19] Bedaso, A., Dejenu, G., & Duko, B., 2022. Depression among caregivers of cancer patients: Updated systematic review and meta-analysis. Psychooncology, 31(11), 1809-1820. Available at: https://www.ncbi.nlm.nih.gov/pmc/articles/PMC9828427/
[20] Ministry of Health, Labour and Welfare (MHLW), 2024. Overview of National Medical Care Expenditures. [online] Available at: https://www.mhlw.go.jp/toukei/list/37-21c.html
[21] Kohno, T., Kato, M., Kohsaka, S., Sudo, T., Tamai, I., Shiraishi, Y., Okuma, Y., Ogasawara, D., Suzuki, T., Yoshida, T., & Mano, H., 2022. C-CAT: The National Datacenter for Cancer Genomic Medicine in Japan. Cancer discovery, 12(11), 2509–2515. Available at: https://www.ncbi.nlm.nih.gov/pmc/articles/PMC9762342/
[22] Tohoku Medical Megabank Organization, 2024. Tohoku University Mega Bank Organization. [online] Available at: https://www.megabank.tohoku.ac.jp/english/

Avoided healthcare costs

	Incident addressable patients (2025-35)	Average avoided healthcare costs per patient	Total avoided healthcare costs (2025-35)
Cell therapy	**67,874** blood cancer patients	**¥1.823M JPY** ($12,000 USD)	**¥123.76Bn JPY** ($0.8Bn USD) total savings
Gene therapy	**2,191** rare genetic disease patients	**¥84.55M JPY** ($556,000 USD)	**¥185.28Bn JPY** ($1.2Bn USD) total savings
Targeted antibody & drug device combination therapy	**1.452M** solid tumour cancer patients	**¥381,100 JPY** ($2,500 USD)	**¥553.57Bn JPY** ($3.6Bn USD) total savings

2.2.2.3 Enhanced skills and expertise for healthcare providers

The rise of precision therapies in Japan will require specialised training for healthcare professionals, enhancing their skills and expertise. Programs like those offered by C-CAT provide clinicians with the tools to effectively use genomic data in treatment planning. Expert panels, which include multidisciplinary teams of oncologists, geneticists, pathologists and bioinformaticians also facilitate continuous learning and enable healthcare providers to make more accurate treatment decisions.

When comprehensive cancer genome profiling tests were added to Japan's reimbursement system in 2019, an expert panel was also formed to guide the interpretation of genomic data.

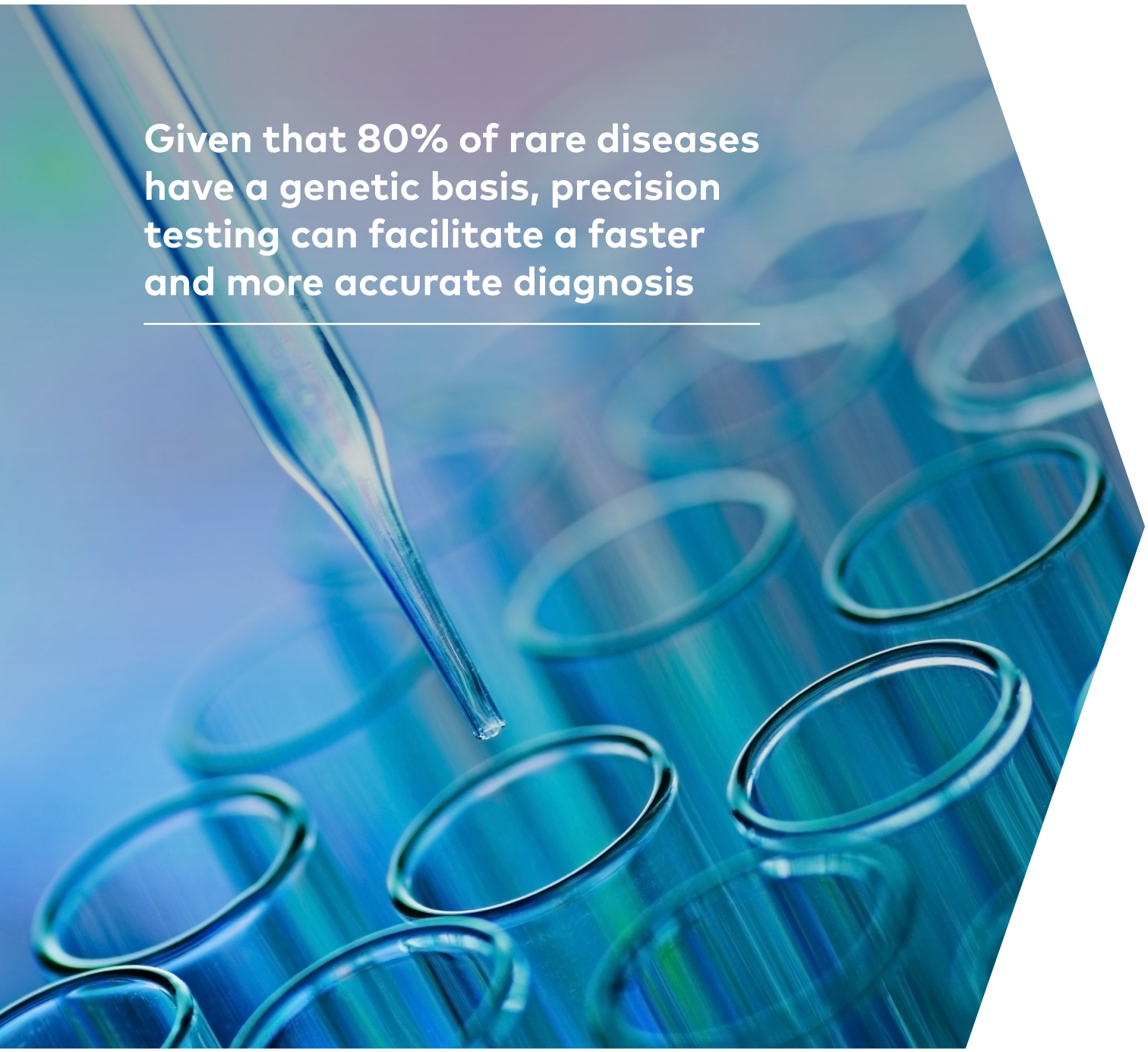
For example, when comprehensive cancer genome profiling tests were added to Japan's reimbursement system in 2019, an expert panel was also formed to guide the interpretation of genomic data. This panel has greatly improved the accuracy of genomic testing and has helped integrate these findings into everyday clinical practice.

2.2.2.4 Improved standard of care and higher efficiency

Precision therapies can improve the efficiency of healthcare by reducing the length of treatment. Studies on cancer patients treated with precision oncology drugs have shown that their average hospital stay is only 3 to 4 days, compared to 7 days for those undergoing chemotherapy.[23]

Precision therapies allows for highly personalised treatment plans, leading to more effective therapies and improved clinical outcomes. By incorporating genetic information and advanced diagnostics into clinical decision-making treatment can begin more quickly, improving overall healthcare efficiency and reducing delays.

[23] Gill, J., Fontrier, A-M., Miracolo, A. and Kanavos, P., 2020. Access to Personalised Oncology in Europe. London School of Economics. Available at: https://doi.org/10.21953/5zsbeehvd3u8



Given that 80% of rare diseases have a genetic basis, precision testing can facilitate a faster and more accurate diagnosis

2.2.3 Economic activity

Improving access to precision therapies can help drive three key economic benefits — greater investment from local and foreign companies across the pharmaceutical value chain, growth in jobs, and increased investment in R&D particularly in clinical trials.

Precision therapies are expected to stimulate significant economic activity in Japan, creating jobs and driving investment in infrastructure needed to develop, manufacture and distribute new therapies.

It is estimated that approximately ¥17Tn JPY ($112Bn USD) will be invested in R&D, manufacturing, sales and distribution of precision therapies.

Between 2025 and 2035, it is estimated that approximately ¥17Tn JPY ($112Bn USD) will be invested in R&D, manufacturing, sales and distribution of precision therapies. This investment could create about 41,000 highly skilled jobs across the pharmaceutical industry, representing a 30% in Japan's current pharmaceutical workforce.[24]

Additional investment in companion diagnostics of ¥2.4Tn JPY ($16Bn USD) will also be required to facilitate access to precision therapies.[25]

As part of R&D, local clinical trials are expected to increase, contributing to market growth of around ¥123 to ¥176Bn JPY ($0.8 to $1.2Bn USD) by 2035. This activity will also provide patients with access to unapproved innovative therapies and help upskill Japan's healthcare workforce.

Pharmaceutical value chain investment and job creation (2025-35)

[24] Ministry of Health, Labour and Welfare (MHLW), 2024. Survey on the Actual Conditions of the Pharmaceutical and Medical Device Industries. [online] Available at: https://www.mhlw.go.jp/toukei/list/87-1.html
[25] Statista, 2024. In-vitro diagnostics - Medical Technology. [online] Available at: https://www.statista.com/outlook/hmo/medical-technology/in-vitro-diagnostics

2.2.4 Multiplier effects

Precision therapies have a far-reaching impact on the Japanese economy, creating ripple effects that boost economic activity across various sectors.

2.2.4.1 Industry economic activity multiplier effects

The economic benefits of precision therapies extend beyond the pharmaceutical sector. Increased investment in the precision therapies sector is expected to have ripple effects across the broader economy. Economic multipliers from direct investment could generate between ¥17Tn and ¥102Tn JPY ($112Bn to $670Bn USD) and support between 41,000 and 247,000 jobs from 2025 to 2035.

These multiplier effects are based on past studies that show how wages from job creation and increased consumer spending generate further economic activity.[26, 27, 28, 29] Tax revenue and job growth in related sectors contribute to this broader economic impact.

Economic multipliers from direct investment could generate between ¥17Tn and ¥102Tn JPY ($112Bn to $670Bn USD) and support between 41,000 and 247,000 jobs from 2025 to 2035.

2.2.4.2 Broader economic multipliers

For the estimated 1.5M patients who could recover or see significant health improvements through precision therapies, these treatments will allow them to return to work sooner and remain in the workforce longer. This will lead to increased productivity and sustainable income tax contributions over the next decade while also boosting consumer spending.

Additionally, about 1.4 to 1.5Mn caregivers will be able to rejoin the workforce or reduce their caregiving responsibilities, further contributing to economic growth.

Additionally, about 1.4 to 1.5Mn caregivers will be able to rejoin the workforce or reduce their caregiving responsibilities, further contributing to economic growth.[30] However, the impact on social productivity improvement is currently limited, given that many precision therapies are only available after the completion of standard of care. It is expected that significant benefits will be realised when precision therapies are used for prevention and early-stage interventions.

[26] Medicines Australia, 2018. The Economic Contribution of the Innovative Pharmaceutical Industry to Australia. [pdf] Available at: https://www.medicinesaustralia.com.au/wp-content/uploads/sites/65/2020/11/Economic-Contribution-Innovative-Pharma-industry-Australia.pdf
[27] Teconomy Partners, 2017. The Economic Contribution of the Life Sciences Sector to the U.S. Economy. [pdf] Available at: http://orclinicalresearch.com/wp-content/uploads/2018/12/teconomy-partners-2017-study.pdf
[28] Australian Commission on Safety and Quality in Health Care, 2017. Economic Evaluation of Investigator-Initiated Clinical Trials Conducted by Networks. [pdf] Available at: https://www.safetyandquality.gov.au/sites/default/files/migrated/Economic-evaluation-of-investigator-initiated-clinical-trials-conducted-by-networks.pdf
[29] Rare Cancers Australia, 2024. The True Value of Investing in Cancer Treatment. [online] Available at: https://www.rarecancers.org.au/news/483/the-true-value-of-investing-in-cancer-treatment
[30] Keio University Hospital, 2024. Radiation Therapy Information and Services. [online] Available at: https://www.hosp.keio.ac.jp/en/annai/raiin/kougaku_seido.html

2.3 Barriers to access

Precision therapies allow for the delivery of the most appropriate treatments by considering factors such as genetic information, medical history, treatment history, and lifestyle. This approach not only improves treatment effectiveness but also helps avoid unnecessary treatments, ultimately contributing to healthcare cost optimization. However, several challenges currently prevent the full implementation of precision therapies, limiting its potential benefits. The key challenges are outlined below:

Barrier to access

2.3.1 Difficulty in reflecting the value of innovative technologies in pricing

Misalignment between the value of precision therapies and current evaluation systems

- Precision therapies can prevent severe diseases and treat them early, based on individual patient characteristics. However, the current medical fee system , especially drug pricing, does not fully account for the value of preventative medicine. This system should also consider cost savings and productivity gains to encourage the development and adoption of precision therapies.

Inadequate consideration of precision therapies' unique characteristics

- The current system uses a standard measure called the ICER (Incremental Cost-Effectiveness Ratio) to assess the value of medical technologies. However, the effectiveness of precision therapies may not be accurately reflected by this alone. Different metrics should be applied based on the specific disease or treatment.

Lack of appropriate experts in evaluations

- Currently, only a limited group of healthcare professionals participate in evaluating medical technologies. To make evaluations more comprehensive, the process should also include experts in health economists, disease-specific specialists, and patient advocacy groups.

Barrier to access

2.3.2 Complex and inflexible insurance system limiting access to CDx/CGP

Lack of compatibility between companion diagnostics and treatment

- Presently, there are 1:1 relationships between diagnostics and treatments. This means that even if a particular biomarker test identifies a promising treatment, patients may not be able to receive it, which limits access to optimal care.
- In addition, once a patient has undergone companion diagnostic testing, if a new drug is released and the patient wishes to undergo testing, they may be forced to pay high out-of-pocket costs because insurance does not cover subsequent tests.

Strict regulation on comprehensive genomic profiling (CGP)

- CGP testing is considered a high-complexity service, restricted to certain advanced medical institutions. As a result, only 60-70% of patients have access to CGP-based treatment. Additionally, CGP is not approved for first-line treatment under insurance, leading to missed opportunities for early treatment and cost savings.
- For example, Capivasertib, approved in 2024 for breast cancer, whose reimbursement is tested by CGP, but CGP is not reimbursed under insurance in front line of treatment, leading to significant out-of-pocket costs for hospitals and a reluctance to use the drug actively.

Barrier to access

2.3.3 Limited access to precision therapies outside of insurance

Challenges in transitioning new technologies to public insurance coverage

- New technologies often need time to demonstrate their value in clinical settings. While 'Mixed Treatment', 'Evaluation-Based Treatment' and 'Patient-Requested Treatment' exist to bridge this gap, they are underutilized due to the heavy burden on medical institutions and lack of public awareness.

Limited use of compassionate access

- Japan lacks systems like the Single Patient Investigational New Drug (IND) available in other countries. Compassionate use is restricted to expanded access trials, which pharmaceutical companies are hesitant to conduct due to the potential impact on approval processes. This limits patients' access to necessary treatments.

Barrier to access

2.3.4 Slow development of new precision therapies[31]

Lack of cross-industry and cross-technology collaboration

- Precision therapies requires collaboration across multiple fields, including AI, biomedical research and real-time health monitoring. However, collaboration between industry, government and academia are not actively promoted in Japan.

Lack of researcher to focus at university hospitals

- University hospitals, which should lead in developing these advanced technologies, do not adequately separate research from clinical duties. Researchers face heavy administrative workloads, preventing them from focusing on their research and innovation. Improving working conditions and reducing administrative burdens are essential to attract and retain talent.

Limited use of medical data

- While Japan is working to improve information sharing between medical institutions, digital transformation is slow. In many cases, test results are still returned on paper under the Clinical Trials Act. Furthermore, the scope of returned on paper and genomic data sharing with pharmaceutical companies is restricted, limiting its use in drug development. Moreover, data aggregated in large biobanks such as the Tohoku Medical Megabank Project is not easily accessible within international frameworks for drug development research, as access is restricted to designated facilities within only Japan.

Weak clinical trial infrastructure

- Slow recruitment for clinical trials, insufficient support functions within hospitals, and limited public interest in clinical research delay the progress of precision therapies in Japan. Improving recruitment process and patient awareness is crucial.

[31] Oncolo, 2024. Oncolo: Cancer Information and News. [online] Available at: https://oncolo.jp

The pace of innovation has outstripped current regulatory reimbursement and pricing processes, which are no longer fit for purpose.

2.4 Recommendations

In this section, we will discuss recommendations for resolving the challenges related to access to precision therapies and enhancing its availability. Precision therapies offer significant value beyond its therapeutic effects, including improvements in healthcare efficiency and economic impact. To accurately evaluate and fully leverage these benefits, it is essential to review and reform existing regulations and healthcare insurance systems. This approach is expected to facilitate the widespread adoption and improved access to precision therapies, thereby benefiting not only patients but society as a whole.

Below are key recommendations:

1. Enhance the regulatory approval systems to better evaluate precision therapies
2. Ease insurance coverage regulations for CDx / CGP
3. Expand access to innovative therapies and compassionate use programs
4. Enhance research infrastructure and collaboration for innovation in precision therapies

Recommendation

2.4.1 Enhance the regulatory approval systems to better evaluate precision therapies[32]

1a. Align regulations and insurance systems with new medical paradigms

- Create a regulatory framework that fully evaluates the patient, social and economic benefits of precision therapies, and ensure that these benefits are reflected in both regulations and the insurance system.

1b. Develop tailored value assessment methods

- Move beyond the uniform reliance on quality adjusted life years (QALY) gains and introduce disease specific indicators that capture the unique value of precision therapies.

1c. Involve appropriate experts in evaluations

- Ensure that evaluations of new medical technologies include experts from clinical settings, patient advocacy groups, and health economists to provide a more comprehensive assessment.
- Case Study Reference: In the COVID-19 Response Subcommittee, a wide range of experts, including infectious disease specialists, public policy experts, healthcare communication planners, labour union representatives, lawyers, and politicians, were involved.[32]

[32] Cabinet Secretariat, 2020. Roster of Members and Temporary Members of the Novel Coronavirus Disease Control Subcommittee, Expert Meeting on Countermeasures against Pandemic Influenza, etc. [online] Available at: https://www.cas.go.jp/jp/seisaku/ful/pdf/meibo-corona.pdf

Recommendation

2.4.2 Ease insurance coverage regulations for CDx/CGP

2a. Promotion of compatibility evaluation for companion diagnostics

- Encourage collaboration among stakeholders such as pharmaceutical companies, diagnostic manufacturers, and academic societies, to advance the compatibility evaluation of companion diagnostics (CDx).
- Ensure more flexible use of precision medicine based on patient needs and clinical judgment.

2b. Relaxation of regulations for CGP implementation

- Revising regulations to allow Comprehensive Genome Profiling (CGP) tests to be used under insurance coverage at an earlier stage based on clinical judgment.
- Introduce measures such as AI-assisted interpretation of CGP test results and the organization of remote expert panels to reduce the burden on the implementation of CGP-based precision medicine.

Recommendation

2.4.3 Expand access to innovative therapies and compassionate use programs

3a. Enhance access to treatment outside of insurance coverage

- Lower the barriers to using innovative treatments outside of public insurance coverage, ensuring that precision therapies can be used based on patient needs and clinical judgement.
- Enhance information provided to patients and healthcare providers regarding new medical technologies, including precision therapies.

3b. Improve access to compassionate use

- Leverage the Single Patient IND (Investigational New Drug) system to enable patients in urgent need of medical treatment to receive therapies earlier. Establish clear guidelines to minimise the potential impact of adverse events on approval applications in compassionate use cases.

Recommendation

2.4.4 Enhance research infrastructure and collaboration for innovation in precision therapies

4a. Promote open innovation

- Foster collaboration and investment environments that break down barriers between industry, government, academia, pharmaceuticals, devices, digital technologies, both within Japan and internationally. Facilitate partnerships that encourage innovation across sectors and borders.

4b. Strengthen the research and development environment

- Separate the functions of research and clinical practice at university hospitals to create a focussed environment for researchers.
- Enhance administrative support for research and improve employment conditions and compensation for researchers to ensure they can concentrate on innovation without administration burdens.

4c. Improve clinical trial recruitment efficiency

- Streamline the roles of medical institutions to consolidate advanced treatment cases and strengthen their capabilities to support clinical trials.
- Improve collection and transparency of clinical trial information. To raise public and patient interest, consider easing regulations on the advertising of medical pharmaceuticals by companies developing and providing precision therapies, and actively promote Patient and Public Involvement (PPI) in medical and clinical research.

4d. Establish medical and genomic data infrastructure

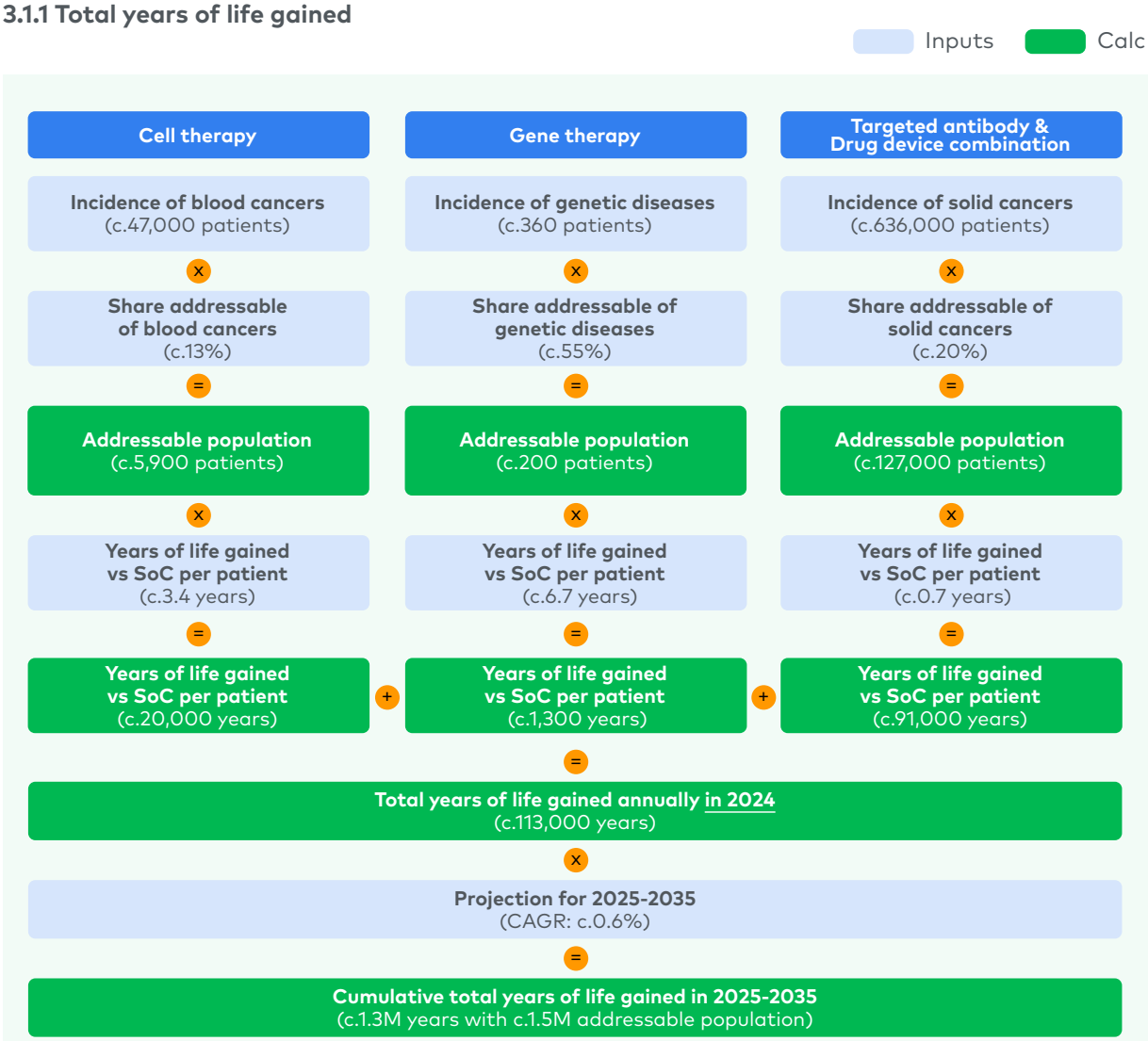
- Remove bottlenecks in promoting digital transformation in healthcare by revising the Clinical Research Act.
- Build a system that facilitates the smooth sharing of patient clinical information, including genomic data, among medical institutions, academic research institutes, and pharmaceutical companies.
- Create a supportive environment for using this data in drug development and clinical research. Additionally, prioritise the recruitment of bioinformatics experts and system engineers in medical institutions to enhance this infrastructure.

3.0 Appendix

This appendix details the approach to quantitative benefits modelling. A range of government data sets, industry data sets, public reports, market reports, and company financial statements have been used as inputs in this modelling. We describe generally how these sources have been used and detail these sources in the bibliography. Advisory committees have reviewed the model methodologies and observed the outputs but were not involved in validating any outputs.

3.1 Modelling methodology

3.1.1 Total years of life gained



Total annual years of life gained were considered for each of the four key technology types — cell therapy, gene therapy, targeted antibody and drug-device combinations.

For cell therapies, targeted antibody and drug-device combinations

- First, the total addressable patient population was estimated by multiplying the incidence population of cancer patients (blood cancers for cell therapies and solid cancers for targeted antibody and drug device combination therapies using the Global Disease Burden database) by the share of those patients addressable by each technology type (estimated using a range of peer-reviewed global studies).

For gene therapies

- The total addressable patient population was estimated by multiplying the incident population of patients with genetic diseases (estimated using peer-reviewed global studies) by the share of patients addressable by gene therapies (based on clinical trial data).
- The annual years of life gained for each therapy was estimated by multiplying the addressable population of targeted therapies by the incremental years of life gained annually (c.113,000 years) over the SoC per patient.
- The incremental years of life gained was sourced from peer-reviewed papers and other HTA reports (e.g., Canadian Agency for Drugs and Technologies in Health (CADTH) assessment report). Currently, many of these treatments for cancer are being used as 3rd or 4th line therapy as they have not yet been tested as an early line therapy. If proven successful for earlier line therapy, years of life gained could be significantly higher.

3.1.2 Avoided healthcare cost

Total avoided healthcare cost in precision therapies was estimated by considering the total annual direct medical cost avoided for cell therapy, gene therapy, and targeted antibody and drug-device combinations. This includes all direct treatment costs, including medicines, hospitalisation etc.

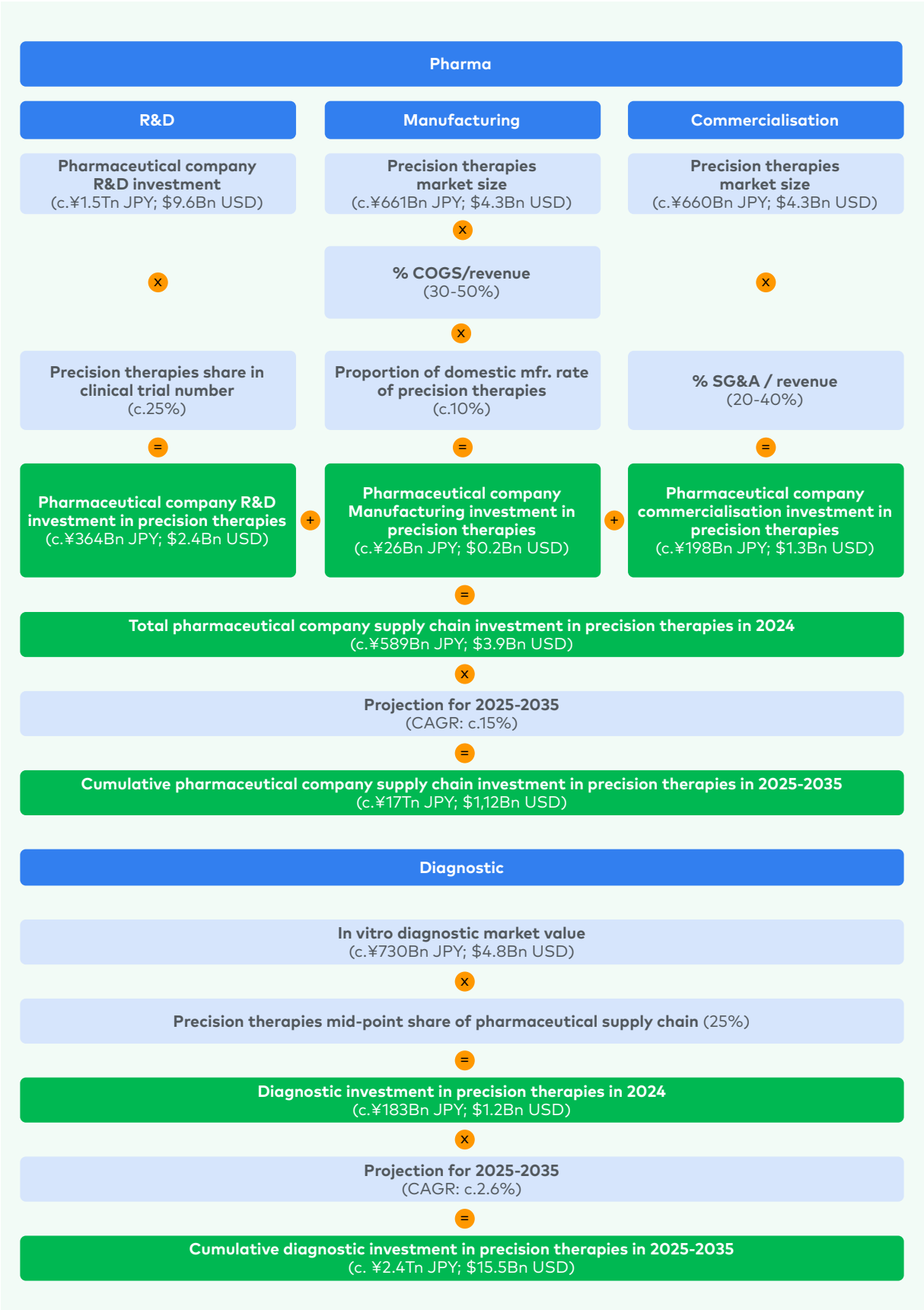
For cell therapies, targeted antibody and drug-device combinations

- First, the total addressable patient population was estimated by multiplying the incidence population of cancer patients (blood cancers for cell therapies and solid cancers for targeted antibody and drug device combination therapies using the Global Disease Burden database) by the share of those patients addressable by each technology type (estimated using a range of peer-reviewed global studies).
- Then we calculated the avoided healthcare cost per patient by multiplying the incremental length of Progression-Free Survival (PFS) per patient realised by 'precision therapies' compared to Standard of Care (SoC) with the average cost of SoC per patient. Both these inputs were derived from peer-reviewed global studies such as clinical trials, government reports and national health expenditure data. Currently, many of these treatments are being used as 3rd or 4th line therapy as they have not yet been tested as an early line therapy. If proven successful for earlier line therapy, Difference in PFS between 'precision therapies' and SOC per patient would likely be even greater.

For gene therapies

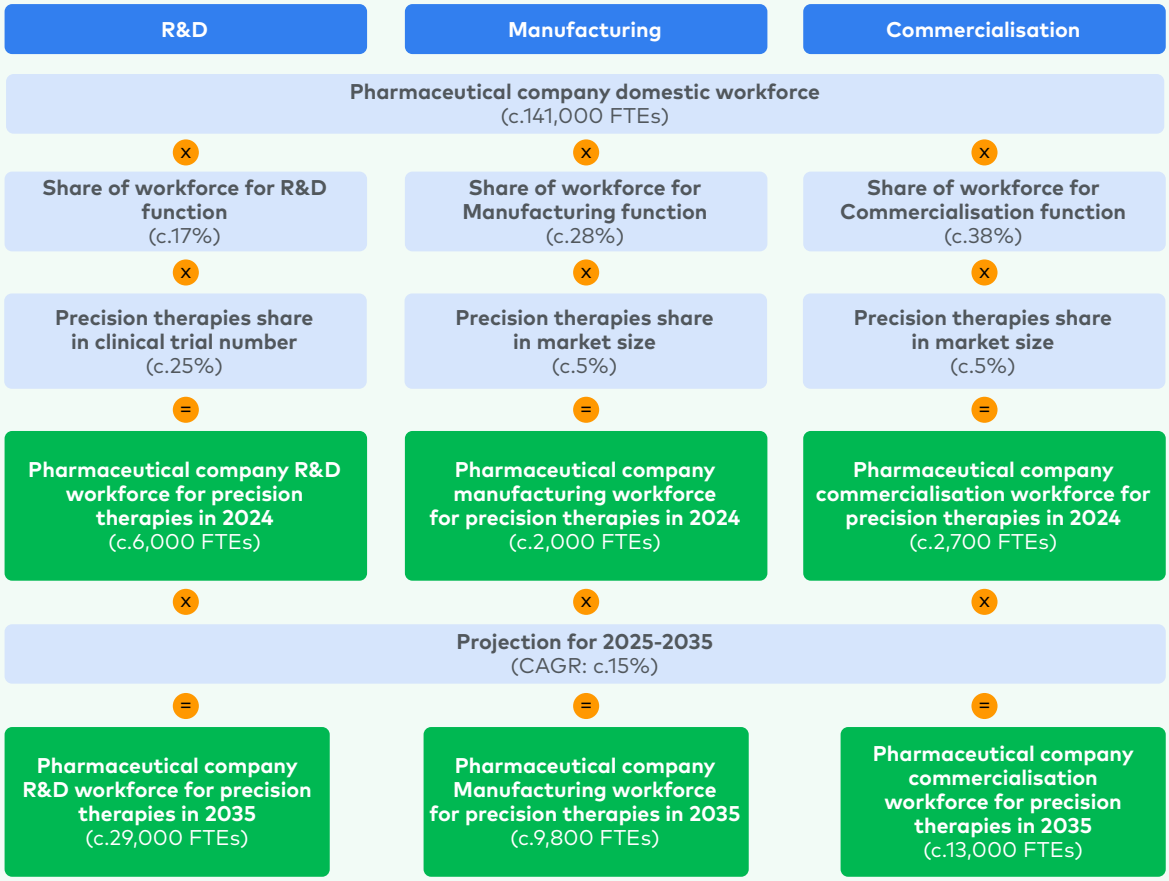
- The total addressable patient population was estimated by multiplying the incident population of patients with genetic diseases (estimated using peer-reviewed global studies) by the share of patients addressable by gene therapies (based on clinical trial data).
- Then we calculated the avoided healthcare cost per patient by estimating the treatment effect duration (given the lack of long-term efficacy data for gene therapies) multiplied by average cost of SoC per patient (estimated using a range of peer-reviewed studies and national health expenditure data).

3.1.3 Industry economic investment



- Total economic investment in precision therapies was estimated by considering the investments in R&D, manufacturing, commercialisation (e.g., sales & marketing, market access, etc.) in 2024 (based on market reports and public investment announcements in news articles).
- R&D investment was estimated by multiplying total pharmaceutical company investment in R&D (from financial reports) in 2024 by the proportion of that investment spent on 'precision therapies' (share of total clinical trials categorised as 'precision therapies' from 2020-23 as a proxy from Pharmaprojects data).
- Manufacturing investment was estimated by multiplying total 'precision therapies' market size in 2024 by an estimated proportion of manufacturing spending (estimated through benchmarking % COGS of listed pharmaceutical companies with a 'precision therapies' focus in Japan) and an estimated proportion of domestic manufacturing rate of 'precision therapies' (estimated though benchmarking domestic manufacturing rate of antibodies).
- Commercialisation investment was estimated by multiplying the total 'precision therapies' market size in 2024 by an estimated proportion of SG&A spend (estimated through benchmarking listed pharmaceutical companies with a 'precision therapies' and oncology focus in Japan using financial reports).
- Diagnostics supply chain investment was estimated based on the total in-vitro market value (based on market reports) and applying of the proportionate weighted average share of 'precision therapies' as applied to the pharmaceutical supply chain.
- The total annual investment values include investments in the supply chain as well as FTE.

3.1.4 FTE



- The total number of jobs supported with precision therapies were estimated by considering jobs supported in R&D, manufacturing, and commercialisation.
- Jobs supported in R&D were estimated by multiplying the total number of employees in the pharmaceutical company in Japan in R&D functions in 2024 (Japan Pharmaceutical Manufacturers Association) and the proportion of jobs supported in 'precision therapies' (which was estimated by the share of total clinical trials categorised as 'precision therapies' from 2020-23 as a proxy using Pharmaprojects data).
- Jobs supported in manufacturing were estimated by multiplying the total number of employees in the pharmaceutical company in Japan in manufacturing function in 2024 (Japan Pharmaceutical Manufacturers Association), and the proportion of the overall pharmaceutical market size attributable to 'precision therapies' (estimated by adding the market size of cell, gene therapies and target antibodies divided by pharmaceutical market size based on market & financial reports) and the proportion of domestic manufacturing rate for 'precision therapies' in Japan in 2015 as proxy.
- Jobs supported in manufacturing and commercialisation were estimated by multiplying the total number of employees in a pharmaceutical company in Japan in those respective functions, i.e., manufacturing and commercialisation in 2024 (Japan Pharmaceutical Manufacturers Association), and the proportion of the overall pharmaceutical market size attributable to 'precision therapies' (estimated by adding the market size of cell, gene therapies and target antibodies divided by pharmaceutical market size).

References

Author(s); Organisation	Title	Year
Australian Institute of Health and Welfare (AIHW)	Australia cancer incidence and mortality books	2017
AIHW	Health expenditure	2024
AIHW	Health system expenditure on cancer and other neoplasms in Australia (2015-16)	2021
AMA	System-wide health sector reform agenda: Medical leaders.	2024
American Society of Gene & Cell Therapy	Vectors 101	2024
Crump, M. et al.; American Society of Hematology	Outcomes in refractory diffuse large B-cell lymphoma: Results from the international SCHOLAR-1 study. Blood	2017
AstraZeneca	Precision medicine	2024
AusBiotech	Australia's Regenerative Medicine Manufacturing Capability & Capacity	2021
Australian Clinical Trials Alliance	Economic evaluation of investigator-initiated clinical trials conducted by networks	2017
Australian Commission on Safety and Quality in Health Care	Economic evaluation of investigator-initiated clinical trials conducted by networks	2024
Cancer Council Australia	Optimal Cancer Care Pathways	2024
Cure Blindness Australia	Retinitis Pigmentosa	2018

Author(s); Organisation	Title	Year
DHAC	Life-saving Spinal Muscular Atrophy medicine to be made available to Australian babies	2020
De Abreu Lourenço, R., et al.; DHAC	Consultation 1 Report. Australian Health Technology Assessment Methods and Policy Review	2023
DHAC	Genomics Health Futures Mission	2024
DHAC	Minister for Health and Aged Care press conference	2024
DHAC	National Health and Medical Industry Growth Plan	2024
DHAC	National Preventive Health Strategy 2021–2030	2021
DoH	$460 million to make Victoria a centre for health, medical research and cancer treatment.	2024
DoH	Accelerating Access to the Best Medicines for Australians Now and into the Future	2024
DoH	Australia's Long Term National Health Plan	2019
DoH	National strategic action plan for rare diseases	2020
European Commission	Rare diseases	2024
Garvan Institute of Medical Research	Precision medicine becomes more accessible for Australians with cancer	2021
Global burden of disease database	Global burden of disease database: Cause of death or injury	2021
IQVIA	The global use of medicines 2024: Outlook to 2028	2024
Johnson & Johnson	Economic & Social Impact in Australia	2018

Author(s); Organisation	Title	Year
Gill, J., Fontrier, A. M., Miracolo, A., & Panos, K.; LSE	Access to personalised oncology in Europe	2020
Memorial Sloan Kettering Cancer Center	Off-the-shelf CAR cell therapy for multiple myeloma shows promise	2024
Mivision	Landmark government funding for Luxturna IRD treatment	2022
National Blood Authority	Australian Bleeding Disorders Registry Annual Report (2019-20)	2020
Bates, S.; NIH	Researchers generate the first complete, gapless sequence of a human genome.	2022
NT Treasury	Population Projections	2024
Omico	The Precision Oncology Screening Platform Enabling Clinical Trials (PrOSPeCT)	2024
Flaherty, C.; OncLive	Quadruplet therapy and early-line CAR T-cell therapy approvals mark shifting standards in multiple myeloma	2024
Kesireddy, M. & Lunning, M.; Oncology	Relapsed or Refractory Diffuse Large B-Cell Lymphoma: "Dazed and Confused"	2022
OR Clinical Research	The Economic Impact of the U.S.	2024
Parliament of Australia	Biopharmaceutical Industry:	2024
Parliament of Australia	2015 National and State	2022
PBS	Public summary document — Onasemnogene abeparvovec	2020
Perkins Institute	Monoclonal antibody facility	2025

Author(s); Organisation	Title	Year
Bedaso, A., Dejenu, G. & Duko, B.; Psychooncology	Depression among caregivers of cancer patients: Updated systematic review and meta-analysis	2022
Cusack, M.B. et al.; RACGP	General practitioners' views on genomics, practice and education: A qualitative interview study. Australian Journal of General Practice	2021
Rare Cancer Australia	Counting the cost: How we can assess the true value of investing in cancer treatment.	2024
Rare Cancers Australia	Belinda trial: CAR-T therapy delivered earlier for relapsed/refractory aggressive non-Hodgkin lymphoma patients	2024
Rare Cancers Australia	The true value of investing in cancer treatment	2024
Rare Diseases Society Singapore (RDSS)	Rare Facts	2024
RArEST Project	National Recommendations for Rare Disease Health Care.	2024
Retina Australia	Retinitis Pigmentosa	2025
Wongchaisuwat, N. et al.; Saudi Journal of Ophthalmology	Retinitis pigmentosa GTPase regulator-related retinopathy and gene therapy	2023
Jackson, S.E. & Trakhtenberg, I.; Stanford University Press	Predictable Winners: A Handbook for Developing, Forecasting, and Launching New Products and Services	2025
Sheykhhasan et al.	CAR-T therapies in multiple myeloma: unleashing the future	2024
Statista	Global spending on precision medicine treatments from 2020 to 2027	2024
Statista	In Vitro Diagnostics — Worldwide	2024
Schofield, D. et al.; The Medical Journal of Australia	The health care and societal costs of inherited retinal diseases in Australia: a microsimulation modelling study	2023

Partner profiles

Yuta Inokuchi

Yuta Inokuchi is a partner & the head of L.E.K. Japan and specializes in healthcare and life sciences. He has extensive experience advising corporates and investors on growth strategy and transactions across pharma, biotech, medtech, diagnostics, CRO/CDMO, and research tools.

Helen Chen

Helen Chen is Global Sector Co-Head for Healthcare and a Greater China Managing Partner of L.E.K. Consulting based in Shanghai. She is also a Director of the firm's Asia Pacific Life Sciences Centre of Excellence. Helen was named one of Consulting magazine's Global Leaders in Consulting in 2019.

Stephanie Newey

Stephanie Newey serves as the head of L.E.K. Australia as well as a coleader of the Australian Healthcare practice. Stephanie has more than 20 years of experience in strategy and consulting, with deep expertise in biopharmaceuticals, life sciences, medtech and digital health.

Manoj Sridhar

Manoj Sridhar is a partner and is dedicated to the firm's Life Sciences and Healthcare practice. Manoj has deep expertise in strategy development, performance improvement and organisational design, and has advised pharmaceutical, medical technology and government clients on a range of strategy and M&A projects in Australia.

Tara Naguleswaran

Tara Naguleswaran is a senior member of the firm's Australia Healthcare Practice.She advises corporate and private equity clients on strategy, M&A, and organisational performance.

LEK™