



How to Ensure the Best Outcome for Patients

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PhARMA
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G20: An Opportunity for Japan to Demonstrate its Global Leadership on Key Health Issues

Global health commitments at the G20 Osaka Summit include:



- Moving towards universal health coverage
- Bolstering primary health care and access to medicines
- Promoting healthy and active aging through the prevention of noncommunicable diseases

Industry Efforts for Patients: Pursuing Innovative Scientific Approaches to Drive Therapeutic Advances

About 8,000 Medicines in Development Globally¹



ALS (Amyotrophic Lateral Sclerosis)

Stem cell therapies aim to replace and/or protect damaged motor neurons and slow disease progression



GENETIC DISORDERS

Gene therapies can halt the progression of many diseases by directly altering the genetic mutations that cause them

RNAi technology can prevent disease from occurring by interfering with the expression of genes



CANCER

Chimeric antigen receptor (CAR) T-cell immunotherapy involves the personalized modification of immune-boosting T-cells to target and kill blood cancer cells

CRISPR/Cas9 technology edits gene sequences in T-cells, reprogramming them to seek and destroy tumor cells

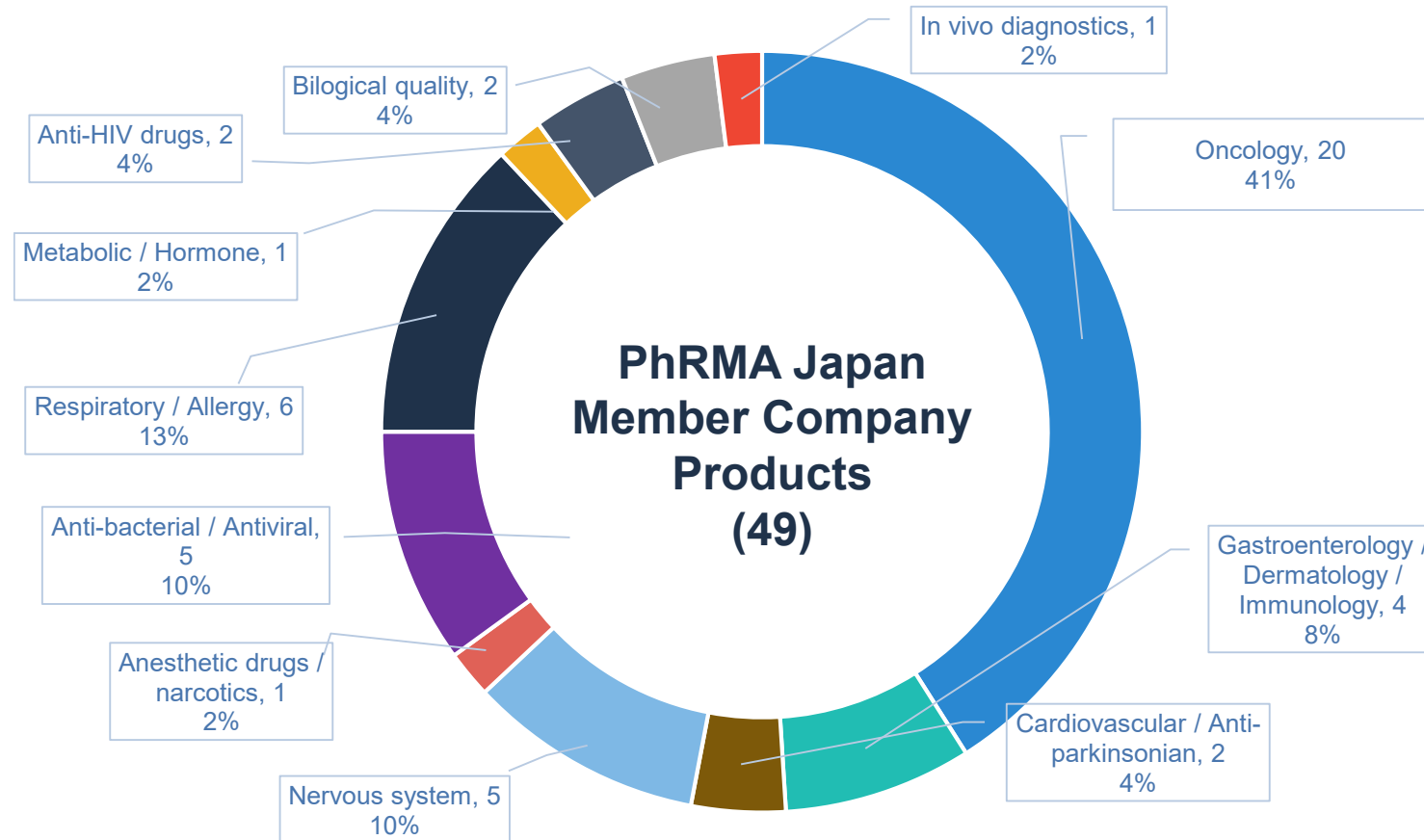
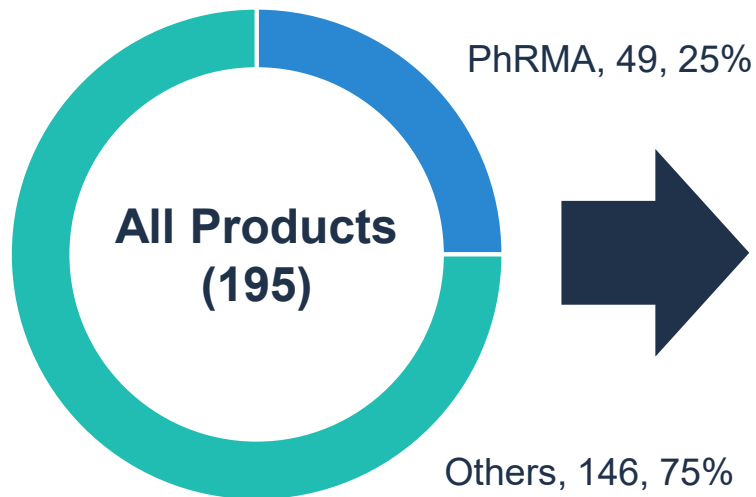


HEMOPHILIA

Adeno-associated viral (AAV) vector-mediated gene therapies enable patients to clot blood and can reduce the need for chronic treatment to prevent bleeding episodes

Industry Efforts for Patients: Bringing New Treatments and Cures to the Market

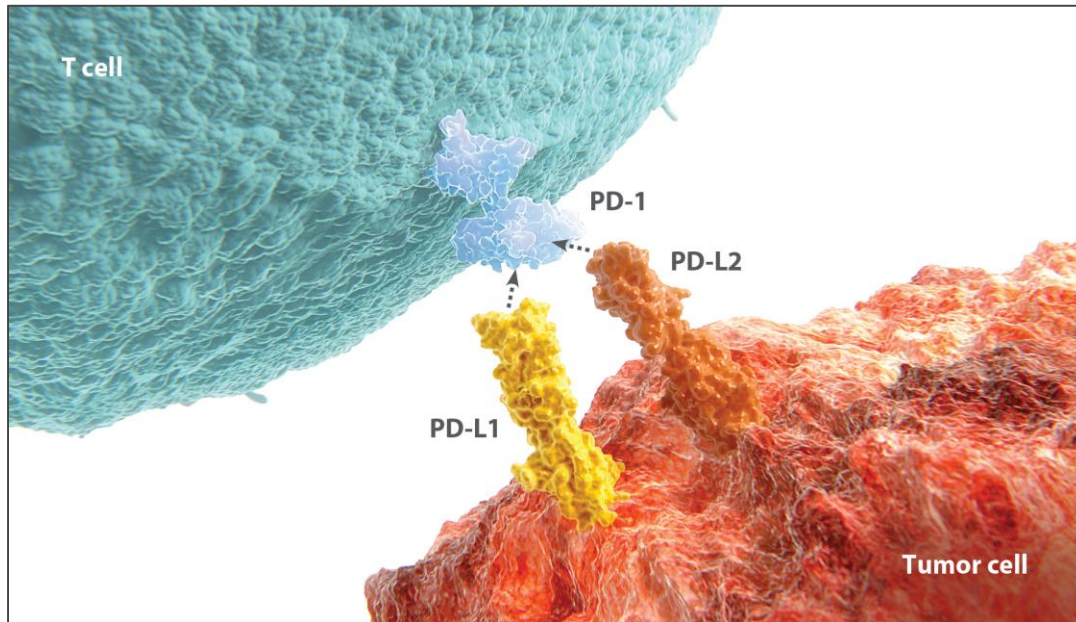
- PhRMA members continue to deliver new and innovative products to Japanese patients
- 25% of all product approvals in 2017 and 2018 were developed by PhRMA Japan's 12 members of which 41% were in the oncology space



Industry Efforts for Patients: Important Advancements in Immuno-Oncology

Pioneering immuno-oncology research has led to new breakthrough cancer treatments and changed survival expectations in multiple tumor types.

Nobel-winning research uncovers ways to activate the immune system to attack cancer



Incorporating Patient Perspectives: The Views of Japanese Patients

Survey reveals Japanese patients have high expectations of the biopharmaceutical industry and of the government and want to ensure they have early access to new medicines.

Patients seek new medicines and vaccines and a seat at the policy-making table

60%

expect pharmaceutical companies to “enhance R&D for new medicines and vaccines”

57%

want to participate in the national policy decision-making process on medicines

Government Efforts for Patients: Legislative Efforts to Improve Patient Access

Recent moves to improve the policy environment could accelerate patient's access to new innovative medicines and incentivize R&D:



Pharmaceuticals and Medical Devices Act Revision

Intractable Disease Law Revision



Immunization Act Revision

Government Efforts for Patients: Radical Reform of the Drug Pricing System

Key elements of the new drug pricing rules raise concern over the direction of reforms:

Price Maintenance Premium

New rules introduced in April 2018 are not science-based and unique in the world

Improve the new PMP rules during the 2020 drug pricing revision

Health Technology Assessment

New HTA system could be used in a way that impedes patient access to new medicines

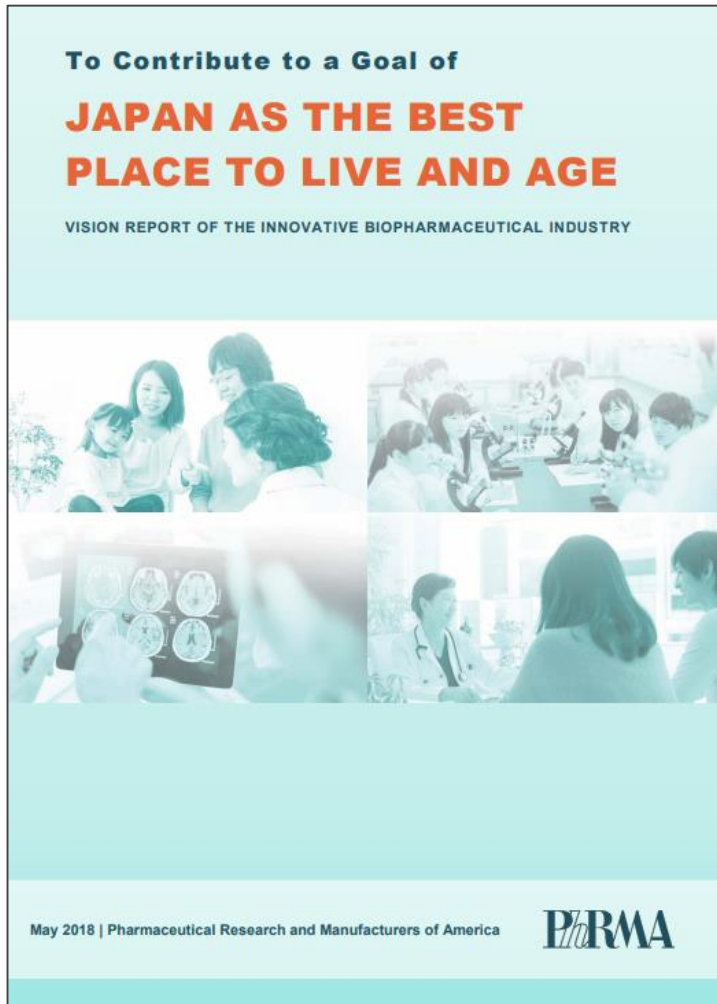
Avoid using HTA for reimbursement decisions

Annual Re-pricing

Annual re-pricing of innovative medicines would negatively impact investment in the Japanese market

Maintain the biennial price revision system for innovative products

PhRMA's Industry Vision



- Provides **constructive proposals** on how the innovative pharmaceutical industry can contribute to making Japan the best place to live and age
- Adopts a **Patients First** approach to reaffirm our commitment to the people of Japan
- Focuses on where we can **best contribute** to the health care system

What the Vision Can Deliver to Japan if Implemented

For Patients

- Continued improvement in health outcomes and healthier lifespans
- Avoid risk of delay in patient access to cutting-edge innovation
- Strengthen inclusion of patient perspectives into their health care

For the Health Care System

- Make possible sustainable investment in innovation
- Increased focus on primary and secondary prevention to significantly reduce NHI expenditures
- Improved efficiency through implementation of data infrastructure and value assessment

For the Economy

- Strengthen domestic efforts to discover and commercialize new medicines
- Help maintain Japan's position as a preferred destination for FDI
- Improved overall productivity and economic growth