How to Ensure the Best Outcome for Patients – 2019 PhRMA's Direction and Initiatives –

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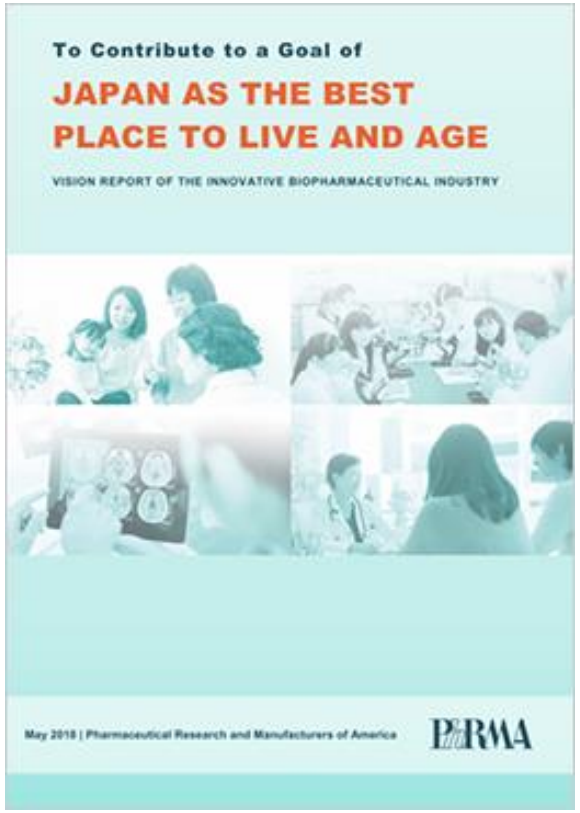
PhRMA
RESEARCH • PROGRESS • HOPE

Agenda

- **Biopharmaceutical Industry Efforts in 2018 and 2019 Outlook**
- **2019 PhRMA Priority Objectives**

Promoting Better Understanding of the Industry from Broad Set of Stakeholders

PhRMA Vision



Researchers Quest

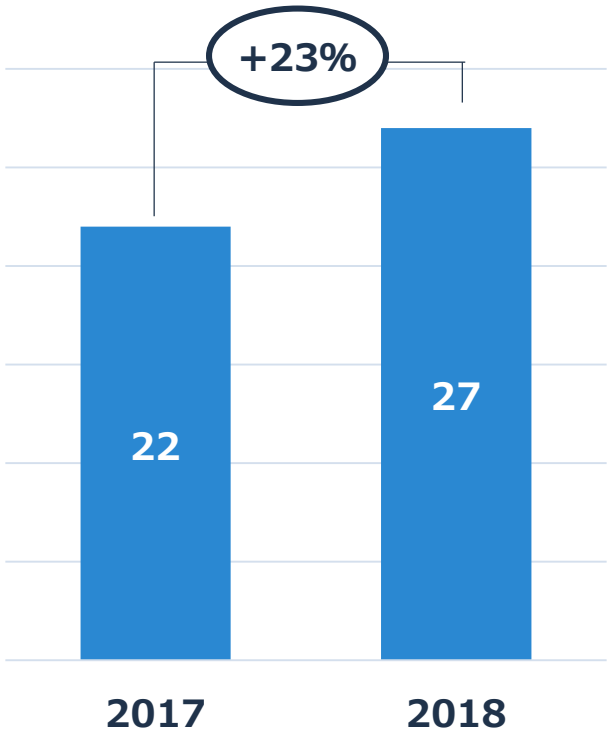
Bringing researchers from around the world to Japan to talk about the exciting pipeline of promising cures that is bringing hope to Japanese patients

Patient Advocacy Academy

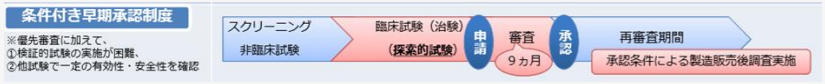
Developing the next generation of patient advocacy leaders by providing them with the knowledge to ensure the patient viewpoint is included in healthcare decision-making

Continued to Deliver Innovative Medicines to Japanese Patients in a Timely Manner

Number of new drug approval by 11 PhRMA member companies



Roll out of conditional early approval system



Approval	Product	Indication	Company
2018/9	LORBRENA	ALK fusion gene-positive unresectable advanced and/or recurrent non-small cell lung cancer resistant or intolerant to ALK tyrosine kinase inhibitors	Pfizer
2018/12	KEYTRUDA	Advanced/recurrent solid tumor with MSI-High progressing after cancer chemotherapy (only when it is difficult to treat with standard treatment)	MSD

Post marketing pharmacovigilance using RWD

MID-NET®
Medical Information Database Network

MID-NET（PMDA's database）
First use by private sector is Pfizer's IBRANCE, using for post marketing database surveillance

Social Security Spending Control Strongly Relying on NHI Price Cuts

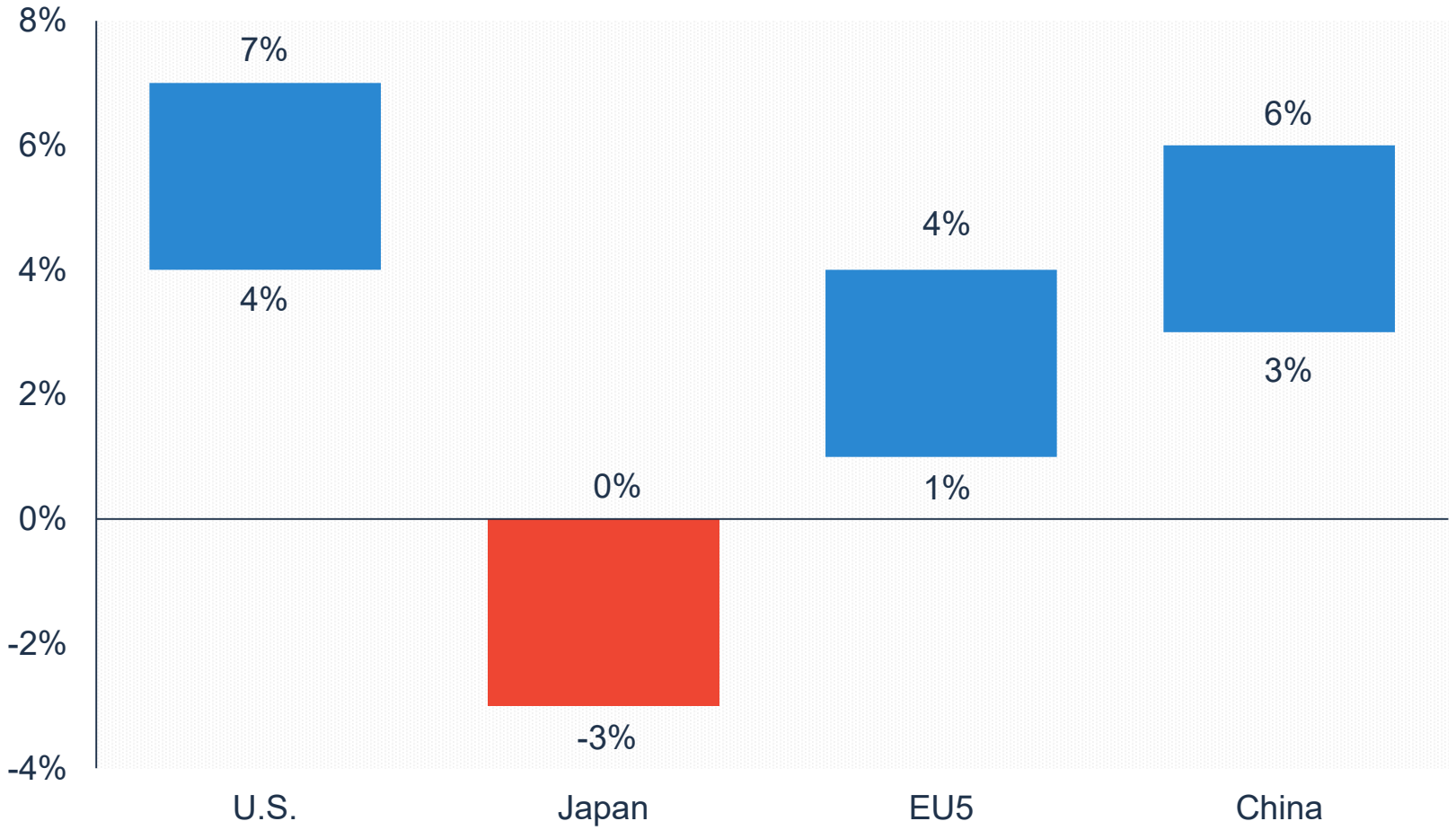
As a market forecast to experience negative growth, depending strongly on cutting drug prices to control social security spending is no longer sustainable

Social security budget over “Intensive Reform Period”

FY Budget	Saving Target	Saving by NHI price revision
2016	– 170 B yen	– 175 B yen
2017	– 140 B yen	– 20 B yen
2018	– 130 B yen	– 176 B yen

More than 80% of total savings generated from price cuts on pharmaceuticals

Japan Forecast to Experience Slowest Growth Among Major Markets



2019-2023 CAGR	
U.S.	4-7%
Japan	(-3)-0%
EU5	1-4%
Germany	3-6%
France	(-1)-2%
Italy	2-5%
U.K.	2-5%
Spain	1-4%
China	3-6%

Source: IQVIA Institute for Human Data Science, *The Global Use of Medicine in 2019 and Outlook to 2023*, January 2019
Note: CAGR = Compound Annual Growth Rate using constant US$ with Q2 2018 exchange rates

Japan's Direction Away from Pro-Innovation Policies

Coincides with China's Efforts to Create a Business Environment that is More Predictable and Rewards Innovation

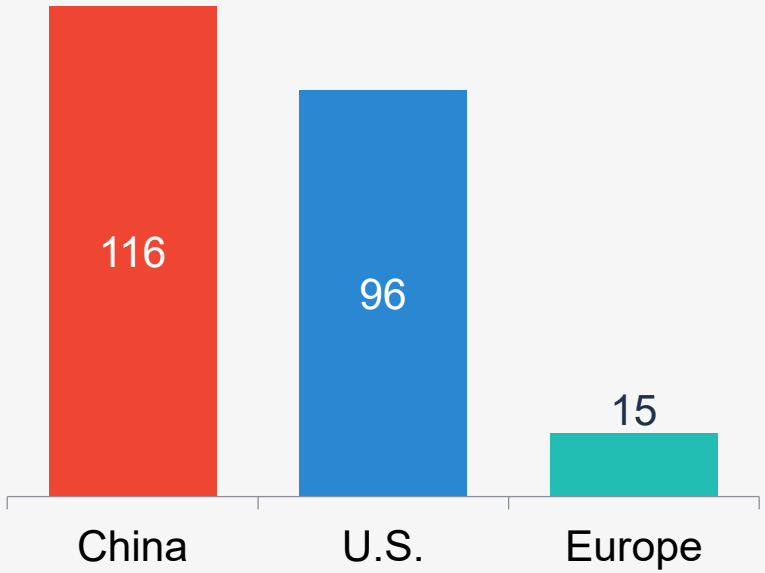
Raising the Standards for Pharmaceutical IP

Significant intellectual property improvements proposed

- China announced plans to provide patent term restoration, patent linkage and up to 12 years regulatory data protection for biologics and 6 years for small molecules
- Meanwhile, Japan has no robust system for patent linkage: it only offers 8 years of regulatory data protection for biopharmaceuticals

Emerging as a World Leader in Cell Therapies

Number of Car-T clinical trials in China now exceeds the US and Europe



Broadening Access for Innovative Medicines

Number of regulatory reforms implemented to facilitate product introduction

- China updated its National Reimbursement Drug List (NRDL) in 2017 following 8-year delay; 36 additional high-value innovative products included
- 17 oncology products added to the NRDL in 2018
- Another NRDL update expected in 2019

Source: ClinicalTrials.gov

Agenda

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Price Maintenance Premium (PMP)

PMP must be revisited at the next revision with the objective of rewarding and promoting innovation appropriately

Deficiencies in the PMP

- Innovation inappropriately defined as speed to market
- Product criteria fails to appropriately evaluate innovativeness
- Company criteria favors large companies over small

Japan's Current Proposal for Full-scale Introduction of HTA is Anti-innovation, Places Patients at Risk

There are significant issues with the proposed system that if not addressed will undermine patient access to innovative medicines

Developed Without Meaningful Stakeholder Input

Developed without meaningful input from global experts, patients and other relevant stakeholders, raising serious questions about the fairness, transparency and predictability of the Japanese market.

Significant Deficiencies in Proposed Appraisal Model

Narrow focus on a cost-per-QALY (ICER) threshold undervalues innovative medicines to patients, providers and the health care system.

Doesn't Reflect Global Best Practices

Disregards key learnings from overseas and departs from recent global trends. Rigorous, continual re-evaluation of this framework is required.

Abbreviations: Cost-effectiveness assessment (CEA), health technology assessment (HTA), quality-adjusted life year (QALY), Incremental Cost-Effectiveness Ratio (ICER).

We Strongly Support the Use of Sound Evidence for Informed Decision-making

Urge further improvement of current MHLW proposal to develop a “best-in-class” HTA system

Describe a sound process
that is open and transparent, with opportunity for input and a strong role for patients and physicians.

Support patient-centered care
by considering patient preferences and heterogeneity, appropriately communicating results, and avoiding misuse.

Deliver reliable, relevant information
by using rigorous, transparent methods that rely on the full range of evidence and prioritize longer-term and broader outcomes.

Value continued scientific and medical progress by accounting for personalized medicine, the step-wise nature of progress, and the inherent value of innovation.

Take a system-wide perspective on value
by examining the full range of tests, treatments, care management approaches and health care services.

Ensuring the Best Outcome for Patients – 2019 PhRMA Priority Objectives –

HTA

- Allow sponsor companies to submit evidence related to non-ICER factors so that experience and examples can be accumulated
- Continue engaging with relevant stakeholders to study possibly appraisal frameworks including our industry proposal
- Recommend that non-ICER factors are incorporated into the appraisal process at the earliest possible timing, and establish a process with multiple stakeholders to develop implementation guidelines

Next Pricing Reform

- PMP reform: expand product criteria, abolish company criteria
- Introduce system to reward innovation by additional indications
- Revisit the modality of repricing
- Secure sustainability through measures such as reform of post-LOE market

Regulatory Reform

- Legislate Sakigake designation and conditional early approval systems; secure international competitiveness in terms of eligible products and number of designated products
- Utilize RWD, ICT to improve efficiency of clinical development and post-marketing pharmacovigilance activities

Ensuring the Best Outcome for Patients
– Long-Term PhRMA Priority Objectives –

To contribute to a goal of Japan as the best place to live and age

INNOVATION PILLAR

Research & Regulation

R&D / Translational research	Guideline harmonization
Expedited approval	Post marketing surveillance

SUSTAINABILITY PILLAR

Investment & Savings

Healthier lifespans	Innovation
Off-patent reform	Prevention

EVIDENCE PILLAR

Quality information

- Data infrastructure
- Value assessment
- Stakeholder involvement

Patient centricity is at heart of what we do and is the foundation of the whole vision