18th DIA Japan Annual Meeting 2021

- New Challenges, New Solutions -

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“PMDA’s Patient Participation Activities”

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DIA

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Agenda

- Patient Participation Activities in Overseas
  - EMA's Activity
  - FDA's Activity
- PMDA's Patient Participation Activities
  - Current Situation
  - Perspective

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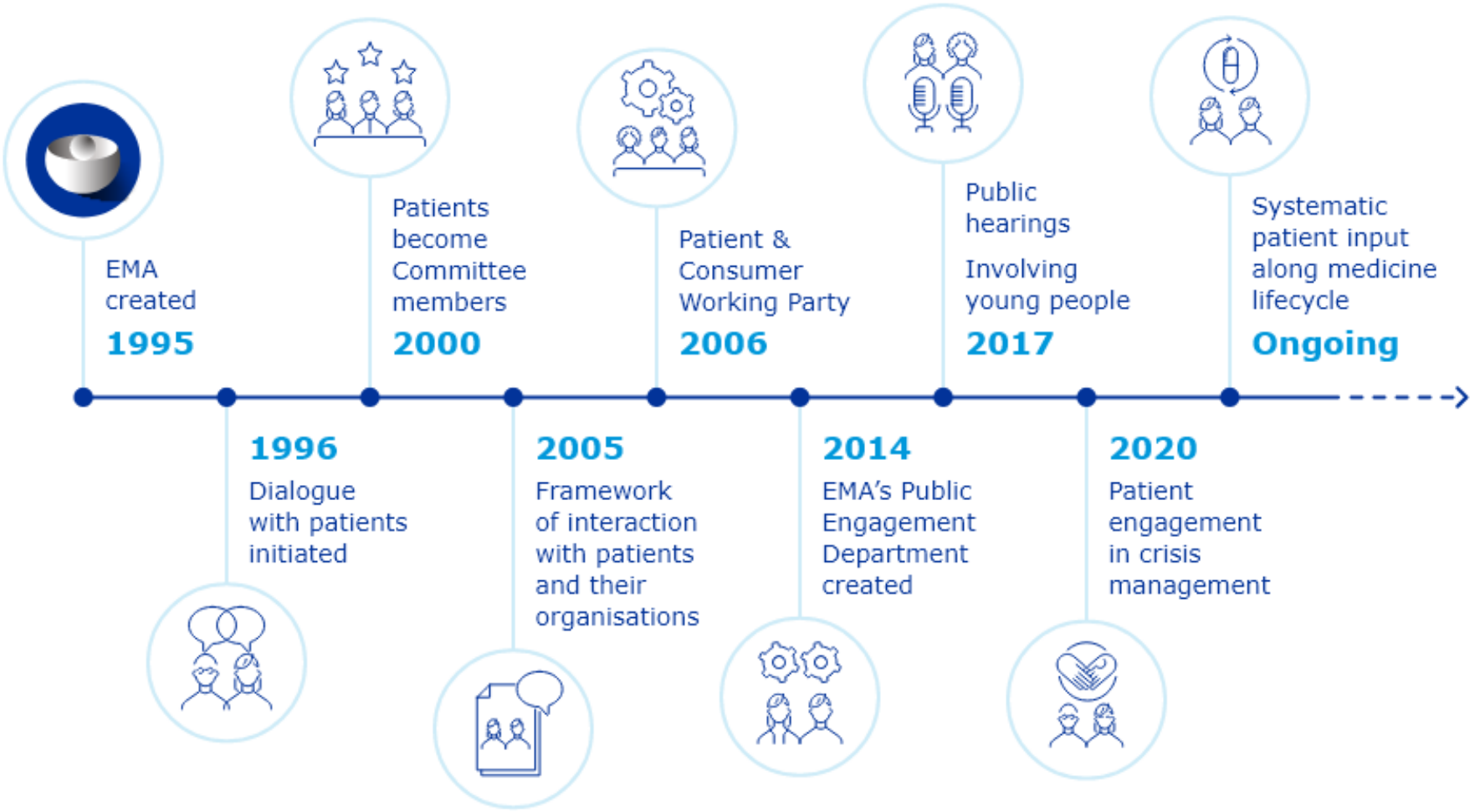
EMA's Path on Patient Participation



https://www.ema.europa.eu/en/partners-networks/patients-consumers（Last access date：Sep 10, 2021）

Drug Life Cycle and Participation in EMA

EARLY DIALOGUE IN MEDICINES DEVELOPMENT

Orphan designation

COMP

PUBLIC SUMMARIES OF ORPHAN DESIGNATION

Advanced therapy classification

CAT

Paediatric investigation plan

PDCO

Scientific advice

SAWP

EVALUATION FOR AUTHORISATION

Marketing authorisation evaluation

CHMP | CAT | PRAC

PACKAGE LEAFLET (NEW)

MEDICINE OVERVIEW

Orphan designation maintenance

COMP

Scientific advisory/ad hoc expert groups

CHMP | PRAC

SAFETY MONITORING OF MEDICINES

Post marketing procedures

CHMP | CAT | PRAC

PACKAGE LEAFLET (RENEWAL)

SAFETY COMMUNICATIONS *

Scientific advisory/ad hoc expert groups

CHMP | PRAC

Public hearings

PRAC

* produced as needed

COMMITTEES & EXPERTS MEETINGS

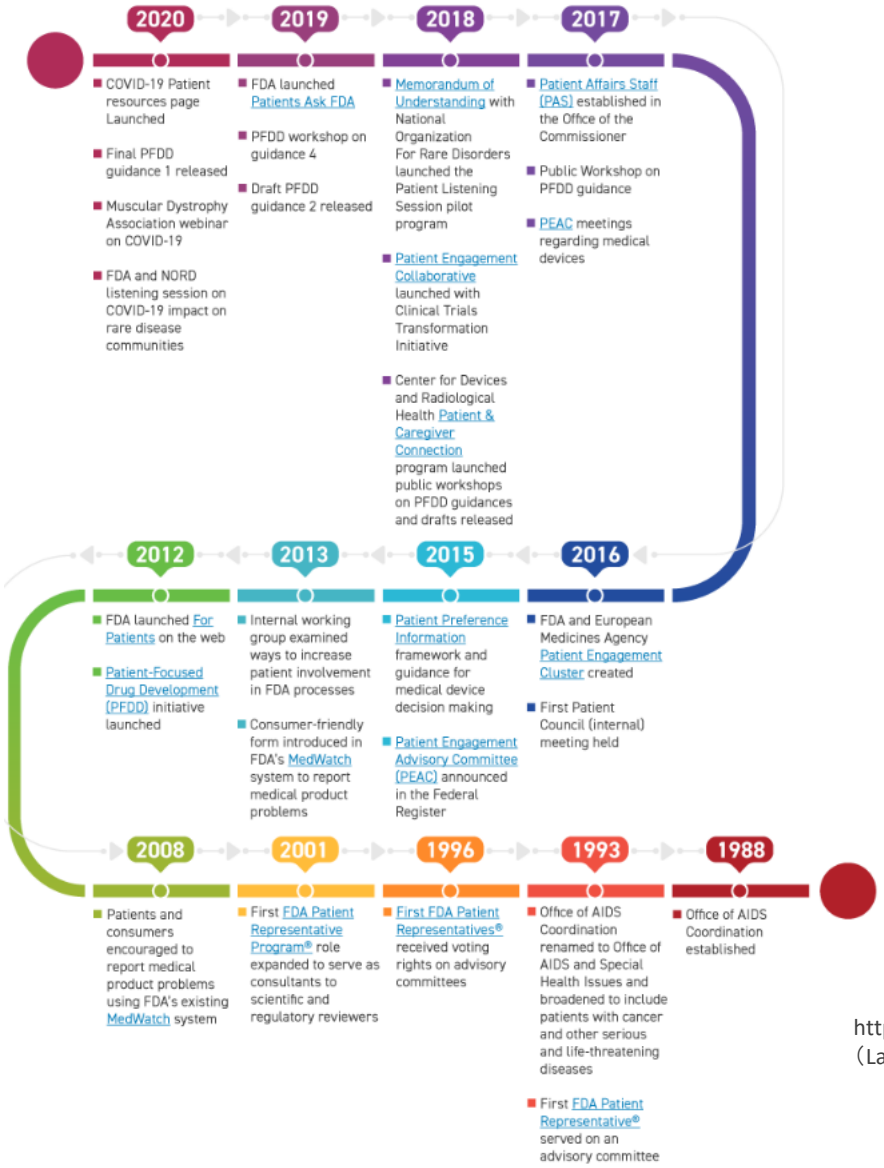
DOCUMENTS FOR THE PUBLIC

COMP: Committee for Orphan Medicinal Products
CAT: Committee for Advanced Therapies
PDCO: Paediatric Committee

CHMP: Committee for Human Medicinal Products
PRAC: Pharmacovigilance and Risk Assessment Committee
SAWP: Scientific Advice Working Party

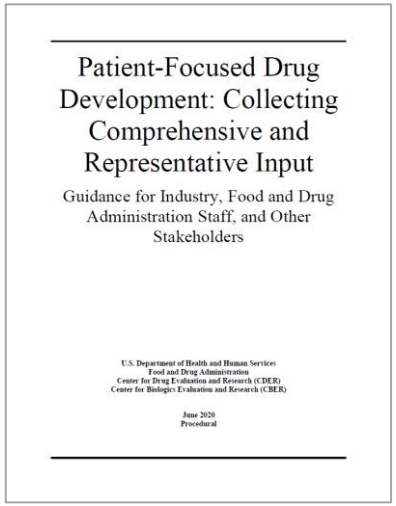
https://www.ema.europa.eu/en/partners-networks/patients-consumers/getting-involved（Last access date：Sep 10, 2021）

FDA's Path on Patient Participation



https://www.fda.gov/patients/evolution-patient-engagement-fda
（Last access date：Sep 10, 2021）

Guidance on PFDD (Patient-Focused Drug Development)

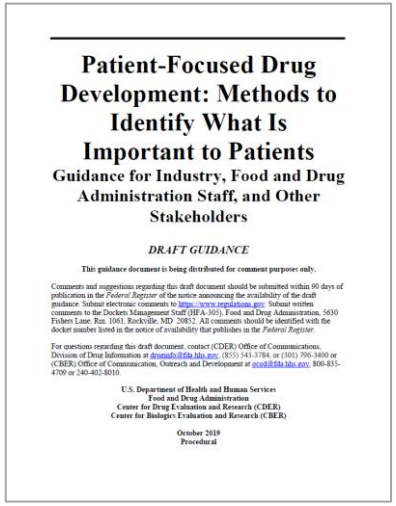


Patient-Focused Drug Development: Collecting Comprehensive and Representative Input

Guidance for Industry, Food and Drug Administration Staff, and Other Stakeholders

U.S. Department of Health and Human Services
Food and Drug Administration
Center for Drug Evaluation and Research (CDER)
Center for Biologics Evaluation and Research (CBER)

June 2020
Procedural



Patient-Focused Drug Development: Methods to Identify What Is Important to Patients

Guidance for Industry, Food and Drug Administration Staff, and Other Stakeholders

DRAFT GUIDANCE

This guidance document is being distributed for comment purposes only.

Comments and suggestions regarding this draft document should be submitted within 90 days of publication in the *Federal Register* of the notice announcing the availability of the draft guidance. Submit electronic comments to https://www.regulations.gov. Submit written comments to the Dockets Management Staff (HFA-305), Food and Drug Administration, 5630 Fishers Lane, Rm. 1061, Rockville, MD 20852. All comments should be identified with the docket number listed in the notice of availability that publishes in the *Federal Register*.

For questions regarding this draft document, contact (CDER) Office of Communications, Division of Drug Information at druginfo@fda.hhs.gov, (855) 543-3784, or (301) 796-3400 or (CBER) Office of Communication, Outreach and Development at ocod@fda.hhs.gov, 800-835-4709 or 240-402-8010.

U.S. Department of Health and Human Services
Food and Drug Administration
Center for Drug Evaluation and Research (CDER)
Center for Biologics Evaluation and Research (CBER)

October 2019
Procedural

- Guidance 1: Collecting Comprehensive and Representative Input
- Guidance 2: Methods to Identify What is Important to Patients
- Guidance 3: Selecting, Developing or Modifying Fit-for-Purpose Clinical Outcomes Assessments
- Guidance 4: Incorporating Clinical Outcome Assessments into Endpoints for Regulatory Decision Making

https://www.fda.gov/drugs/development-approval-process-drugs/fda-patient-focused-drug-development-guidance-series-enhancing-incorporation-patients-voice-medical
（Last access date：Sep 10, 2021）

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Patient Centricity Working Group within PMDA launched

- Background
  - ✓ An internal working group on patient engagement was set up within PMDA, consisting of PMDA staff involved in pre-market review and post-marketing.
  - ✓ PMDA will explore the activities to share challenges from the patient‘s perspective and communicate with patients, regarding drug development and safety measures.
- Launched May 2019
- Goals
  - ✓ Optimize the way of patient engagement to drug development and safety measures
  - ✓ Develop guidance on the relationship between patient activities and PMDA

History to reflect :
Requests from Patients on Drugs

- 2005 - 2009 Committee on Evaluation of Unapproved Drug Use Issues
- 2009 - Committee on Evaluation of Unapproved drug / off-label use drug review with high medical need

These committees have evaluated medical necessity in Japan about drugs. That has been already approved Europe and the United States but not approved in Japan, based on requests from patients and professional societies of medicine. MHLW has encouraged companies to develop necessity drugs referring outputs from committees.

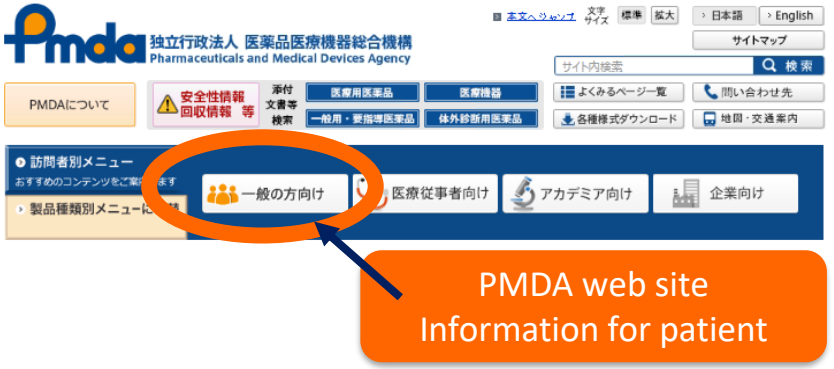
Examples of output:
Bevacizumab for ovarian cancer
Oxaliplatin for gastric cancer
Bendamustine for chronic lymphocytic leukemia, etc

- 2016 - Additional pathways were started to access unapproved drugs and off-label drugs: "Clinical trials conducted from a humanitarian point of view" and "Patient-offered medical treatment"

Other activities of PMDA / MHLW to achieve "Patient Participation"

1. Information provision for patients

- Drug Guide for Patients (2005-)
- Serious Adverse Reactions Manual for patients (2005-)
- Delivery of Safety information for patients (safety alert, request for appropriate use, etc.)



2. Information provided by patients

- Adverse Event reporting system from patients (trial: 2012-, officially operated: 2019-)
- Committee on Evaluation of Unapproved drug / off-label use drug review with high medical need (2009-)
- Other request form etc.

3. Patient participation in regulatory advisory meetings

- MHLW Pharmaceutical Affairs and Food Sanitation Council
- PMDA Management Council, etc.

4. Other

- Construct unified registry of clinical trials to improve access to clinical trials for patients (2018-)

Interactions between regulators and patient groups (examples)

- **SMA (Spinal Muscular Atrophy) Family Association**

  Patient group met PMDA Chief Executive during Zolgensma review for exchanging views and requesting early approval

- **Japan Mucopolysaccharidosis Patient Family Association**

  submitted a request to the Ministry, regarding “SAKIGAKE” designation system for the treatment of mucopolysaccharidosis type II (Hunter syndrome)

Reference : https://www.sma-kazoku.net/, http://www.mps-japan.org/

Patient Centricity Working Group Interaction with Patient Groups (example of education opportunity)

Exchange of opinions with members of the certified NPO Consumer Organization for Medicine & Law (COML)

- Patient Centricity WG introduced the major activities, operations and regulations of PMDA for drug review, safety, and ADR relief.
- Active exchange of opinions on PMDA's activities from the patient's perspective

External resources to leverage

- **Guidance from Japan Pharmaceutical Manufactures Association**
  - “Drug development utilizing the voice of patients-Patient Centricity by pharmaceutical companies-”（June 2018）
  - “A guidebook for pharmaceutical companies to carry out activities based on Patient Centricity-Drug development that utilizes the voice of patients-”（September 2019）
  - “Drug development that utilizes the voices of patients Communication guidebook for pharmaceutical companies to promote activities based on patient groups and Patient Centricity”（September 2019）
- **Japan Agency for Medical Research and Development(AMED)**
  “Patient and Public Involvement (PPI) Guidebook-As the first step toward collaboration between patients and researchers-”（March 2019）

PPI Activities in Japan

PPI Consortium in Japan

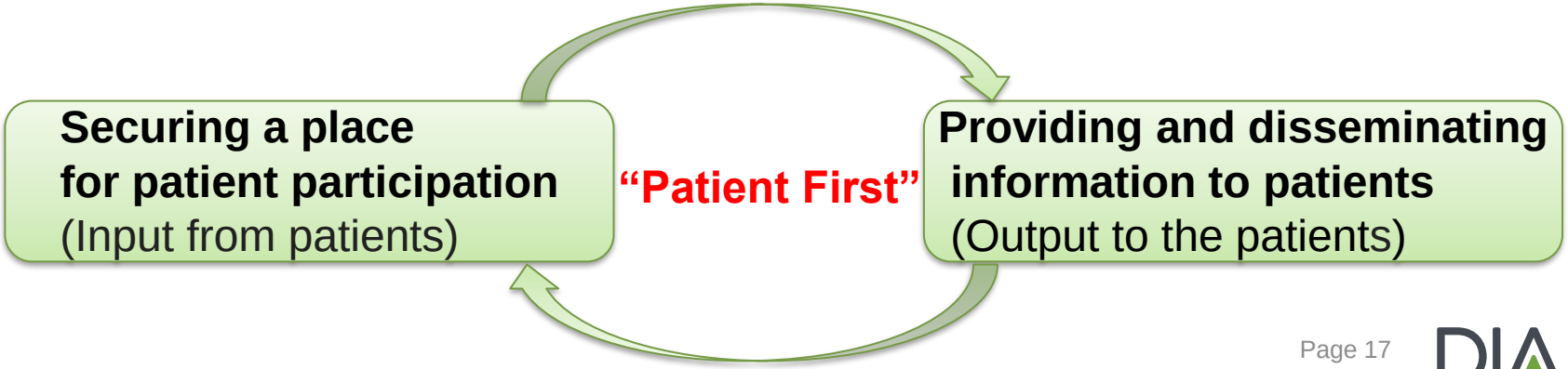
- Established in July, 2019 as the open forum of patients, industry, academia and regulatory authority
- Partnering with EUPATI to introduce EUPATI tools to patient and public

PMDA started to collaborate with PPI consortium for providing EUPATI tools in Japanese.

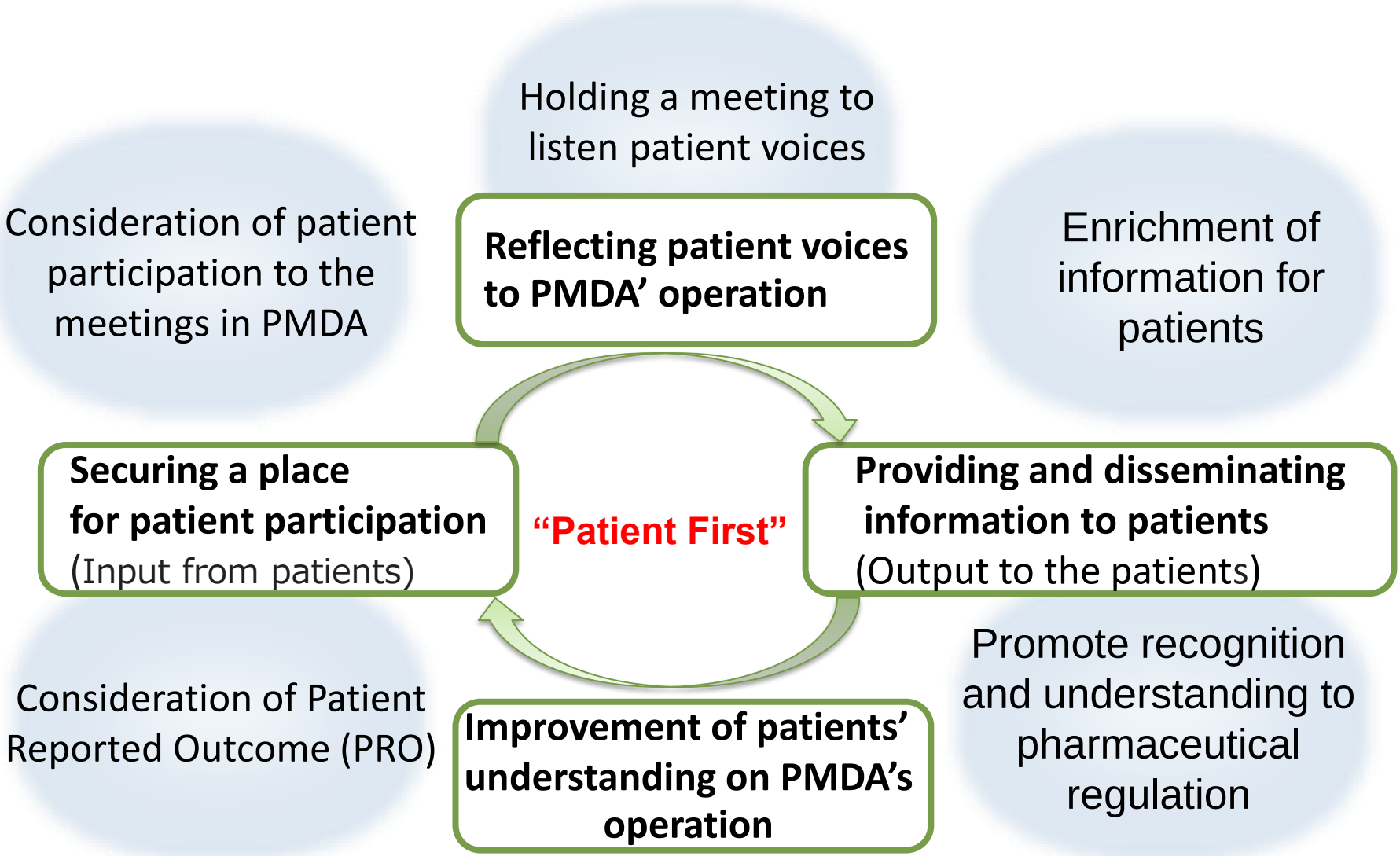
Patients Centricity Activities of PMDA - Principles

- We aim to achieve the PMDA's policy of "Patient First" by reflecting the voices, opinions, and experiences of patients, and to improve their understanding and satisfaction with pharmaceuticals and medical devices.

- Development of new guidance that stipulates principles that PMDA executives and employees should refer to in promoting patient participation in PMDA's operations.
- Basic Concept of the guidance
  - The PMDA's policy of "Patient First" will be achieved by listening patient voices.
  - To collect information of patient voices effectively, information for patients should be enriched to enhance patients' understanding of PMDA's operations and pharmaceutical regulations.
  - Promote patient involvement through both of these efforts.



Patient Centricity Activities of PMDA – Challenges



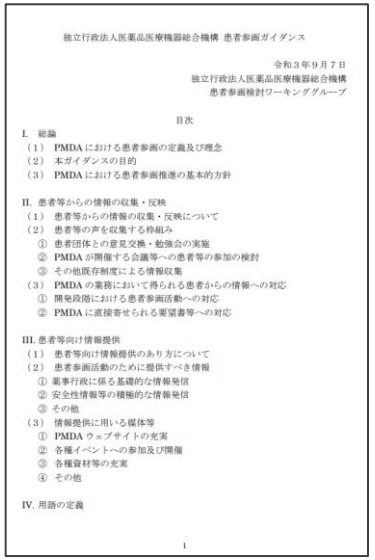
Pharmaceuticals and Medical Devices Agency Guidance on Patient Participation

I. Overview
- Definition and Philosophy of Patient Participation in PMDA
- Purpose of this guidance
- Basic Policy for Promoting Patient Participation at PMDA

II. Collection and reflection of information from patients
- Collection and reflection of information from patients, etc.
- A framework for collecting the voices of patients, etc.
- Responding to patient information from PMDA operations

III. Provision of information to patients, etc.
- A method of providing information to patients, etc.
- Information to be provided for patient participation activities
- Media used for information provision, etc.

IV. Definition of terms



※ An English version is scheduled to be released in a few days