第9回DIAクリニカルオペレーション・モニタリング ワークショップ

COVID-19を経験した我々がこれからやる事、変える事

2021年7月8日（木）～ 10日（土）| Zoom Webinar

PMDAにおける患者参画検討WGの取組み
“PMDA’s Patient Participation Activities”

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Agenda

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

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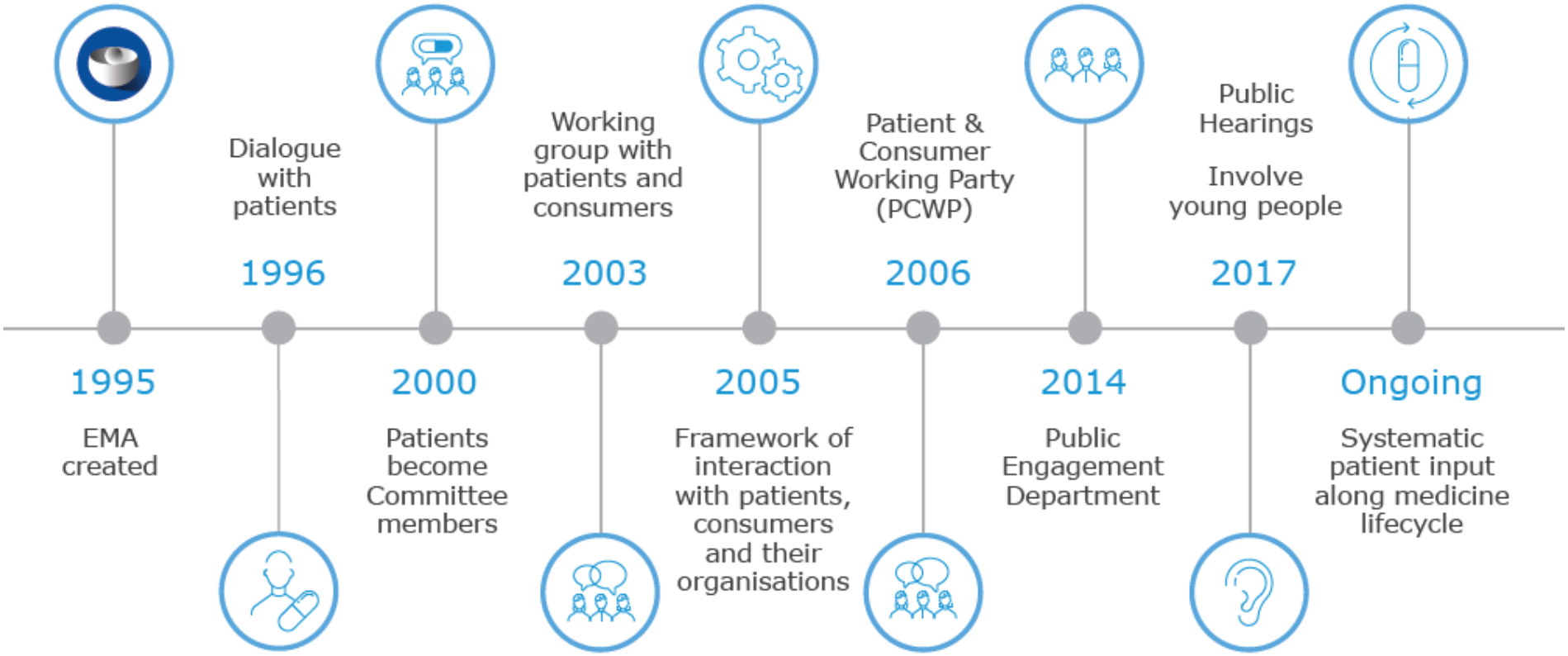
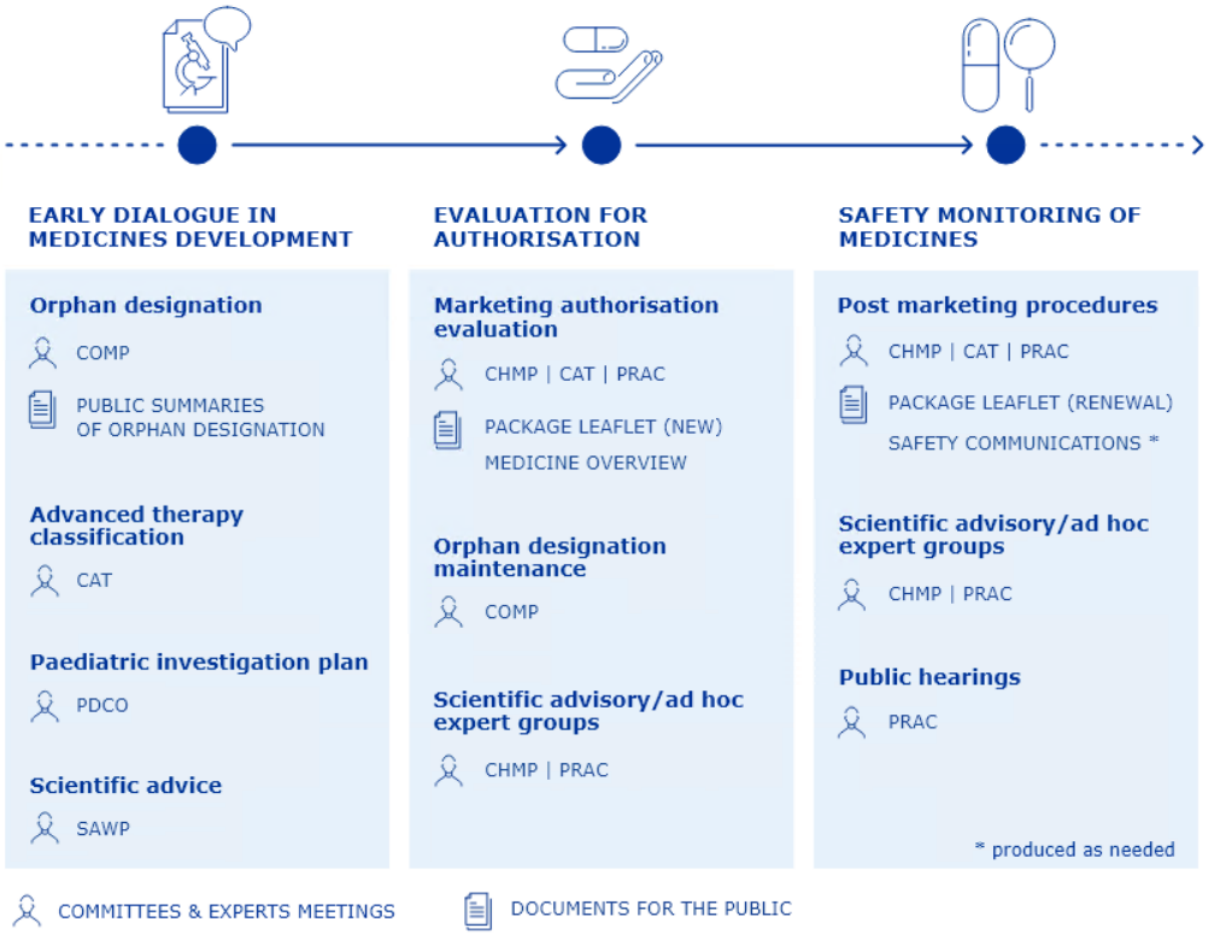
EMA's Path on Patient Participation



https://www.ema.europa.eu/en/partners-networks/patients-consumers, Accessed July 2021

Drug Life Cycle and Participation in EMA

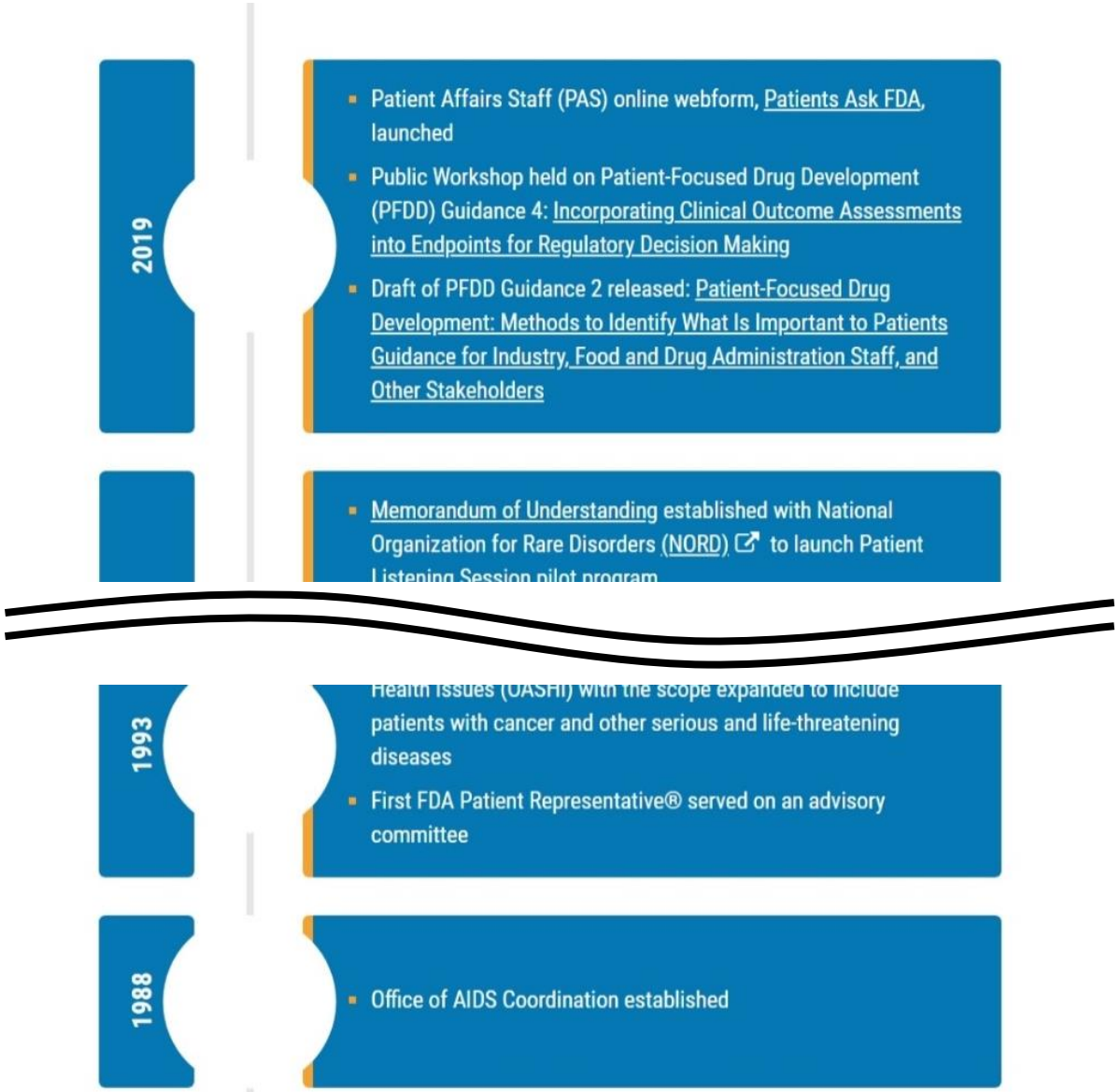


COMP: Committee for Orphan Medicinal Products
CAT: Committee for Advanced Therapies
PDCO: Paediatric Committee

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

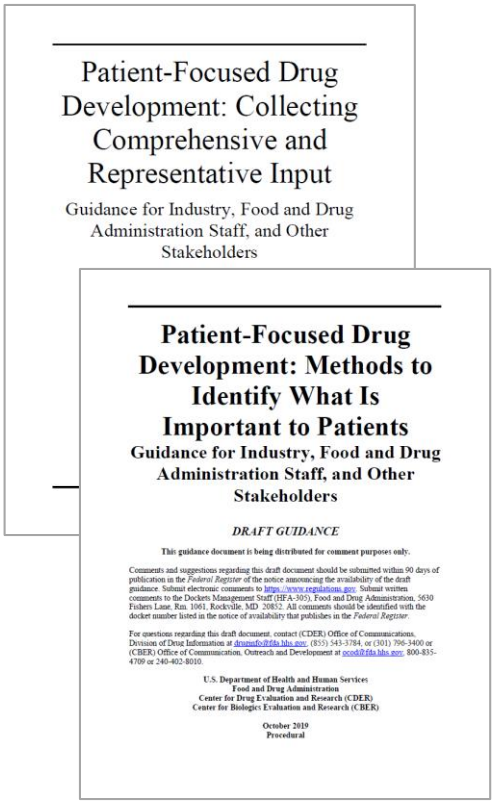
https://www.ema.europa.eu/en/partners-networks/patients-consumers/getting-involved, Accessed July 2021

FDA's Path on Patient Participation



https://www.fda.gov/patients/learn-about-fda-patient-engagement, Accessed July 2021

Guidance on PFDD



- 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, Accessed July 2021

CIOMS's Activity: Working Group XI Patient Involvement

- The CIOMS Working Group XI on patient involvement in the development and safe use of medicines was launched in April 2018.
- It includes participants from patient organizations, industry, regulators, academia and the World Medical Association.
- These experts are working together to formulate pragmatic Points to Consider in patient involvement.
- The guidance will provide a comprehensive overview of present knowledge and existing initiatives, and will address a wide range of the remaining challenges and practice gaps.
- The optimal consideration of patient perspectives will support the safe and effective use of medicines, thereby helping to improve the health of individuals and the public.

https://cioms.ch/working-groups/working-group-xi-patient-involvement/, Accessed July 2021

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“ 4Fs (Firsts) ” Policy

- **Patient First**

  We make good communication with healthcare professional and work giving the highest priority to patient satisfaction.

- **Access First**

  We accelerate the access to innovative medical products of public demand, while ensuring appropriate risk-benefit balance.

- **Safety First**

  Focusing on risk mitigation, we implement efficient post-marketing data collection and product distribution control, especially for accelerated approval schemes such as “Conditional Early Approval System” and “SAKIGAKE Designation System”.

- **Asia First**

  We promote regulatory harmonization and improve public health among Asian countries/regions, as well as build trust in Japanese regulatory system.

Patient Centricity Working Group within PMDA launched

- Purpose
  - ✓ 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 the challenges from the patient‘s perspective and communicate with patients, regarding drug development and safety measures.
- Launched　May 2019
- Goals

  PMDA is working on patient participation and collaboration :
  - Optimize the way of patient engagement
  - Develop guidance on the relationship between patient activities and PMDA
- Guidance and public information will be announced in two years.

Patient Participation of Drug Access Improvement process : Requests From Patients

- 2005 - 2009 Committee on Evaluation of Unapproved Drug Use Issues
- 2010 - Committee on Evaluation of Unapproved Drug / off-label use Drug with high medical needs

For drugs that have been approved in Europe and the United States but are not approved or indicated in Japan (unapproved drugs / off-label drugs), the medical necessity is evaluated, based on the requests from professional societies and patients. The committee aimed at encouraging pharmaceutical companies to develop these products in Japan.

Examples of output:
Avastin for ovarian cancer
Elplat for gastric cancer
Treakisym for chronic lymphocytic leukemia, etc

- 2016 - Access system for unapproved drugs and off-label drugs: "Clinical trials conducted from a humanitarian point of view" and "Patient-offered medical treatment" systems started

A system that provides unapproved drugs, etc. from a humanitarian point of view to patients who do not meet the criteria for participation in clinical trials.

Patient Participation Activities so far

1. Information provision for patients

- Drug Guide for Patients (2005-)
- Manual for Serious Adverse Reactions and Diseases (2005-)
- Provision of other information for patients (safety alert, request for appropriate use, etc.)

PMDA web site
Information for patient

2. Information provided by patients

- Patient Adverse Event reporting system (trial: 2012-, formalization: 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 the regulatory advisory meetings

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

4. Other

- Access to registration information for participating in clinical trials (2018-)

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

Centricity Activities of PMDA – Principles

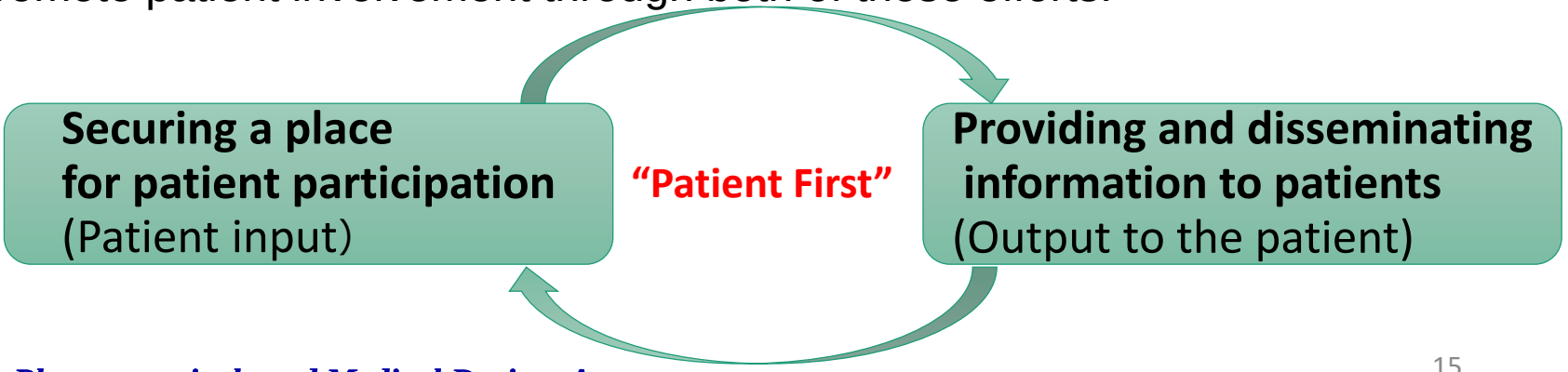
- We aim to achieve the PMDA's policy of "Patient First" by reflecting the voices, opinions, and experiences of patients, and to improve their understanding and satisfaction with pharmaceuticals and medical devices.

- Develop 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 (draft)

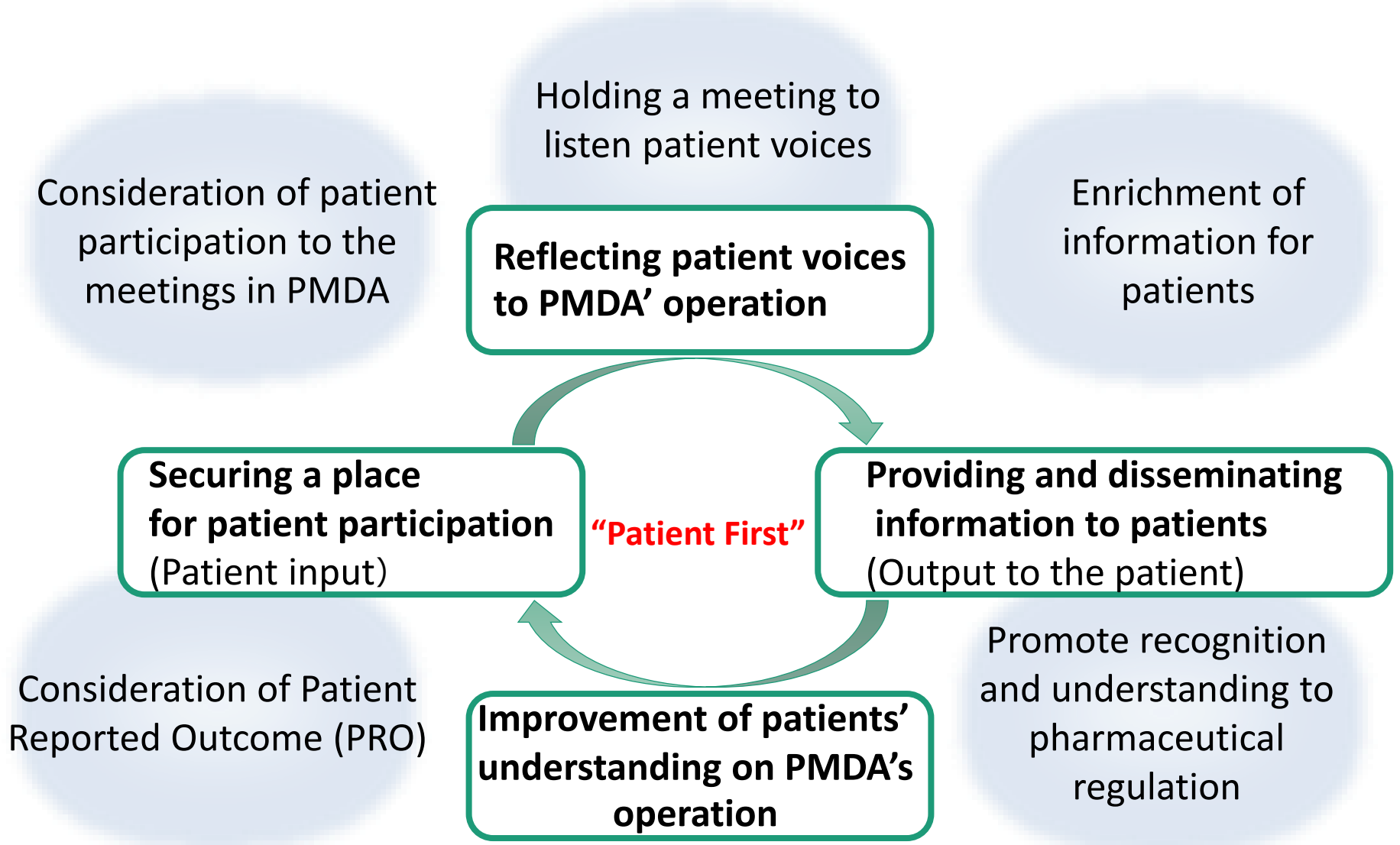
  - The PMDA's policy of "Patient First" will be achieved by listening patient voices.

  - To collect information of patient voices effectively, information for patients will 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



Patient Centricity Activities of PMDA – Challenges

- **Patient Reported Outcomes (PROs)**
  - Seeking more systematic ways of collecting patient experiences for drug evaluation (developing PMDA guidance)
  - Patient experiences are to be reflected in clinical trial designs, by sponsors and in PMDA consultations.
  - In the mean time, we will be facing PROs in multi-regional clinical trials (MRCT), considering how to interpret patient experiences and outcomes under Japanese cultural and clinical environment for enrolling Japanese patients.
  - We have not experienced any serious scientific discrepancy so far.

……We are following developments of USFDA for PFDD

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 Consortium in Japan（founded in July 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.