Promoting Access to Better Innovative Drugs

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Asia Partnership Conference of Pharmaceutical Associations

23rd April 2024

Today's Topics

1. 5th Mid-term plan (FY2024–FY2028)
2. PMDA's efforts to improve access to better innovative products
3. PMDA's international cooperation in Asia

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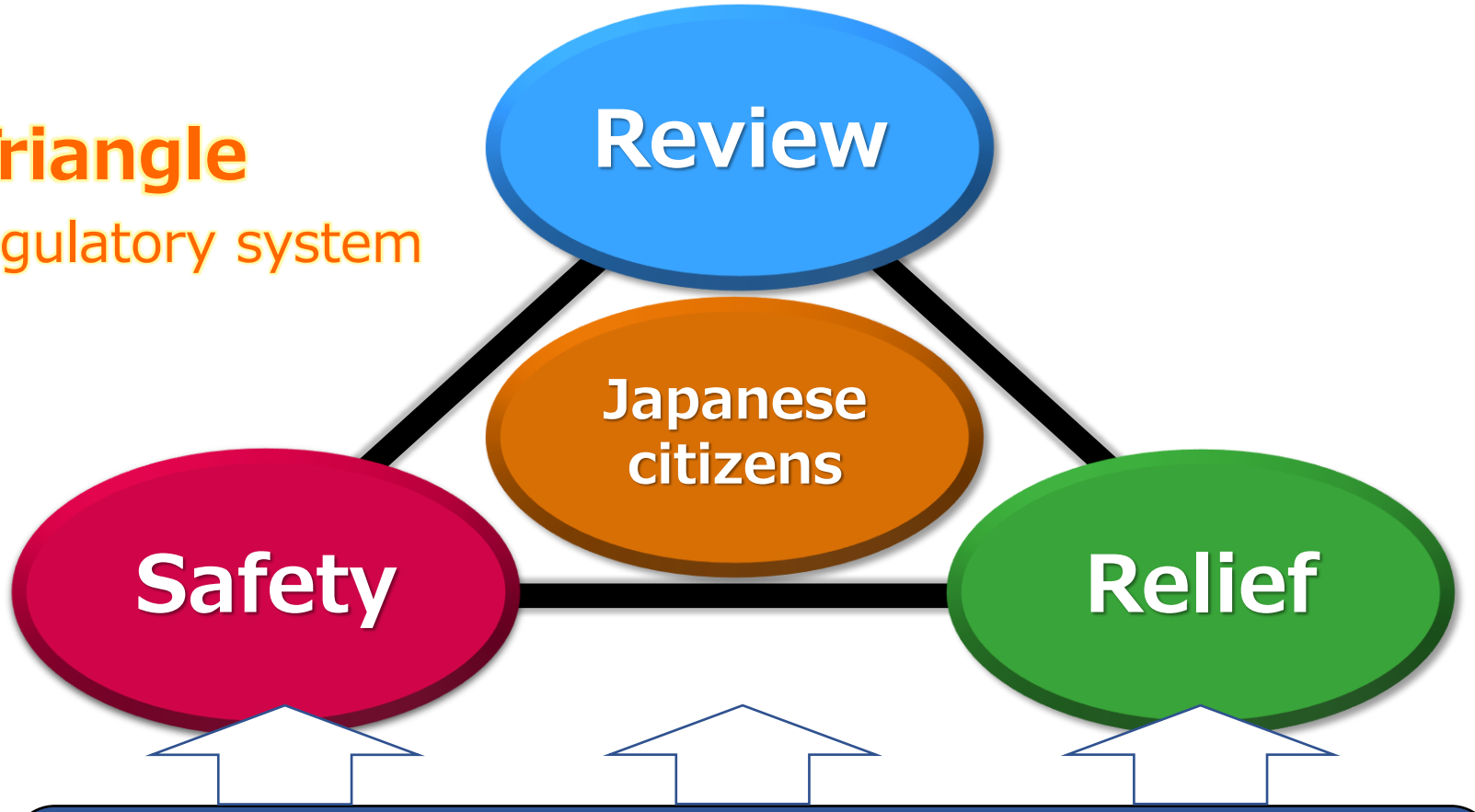
Basic structure for 5th Mid-term plan

Safety Triangle

For world-class regulatory system



- For further “Quality” through Regulatory Science
- For Strategic international activities
- Governance and recruit professional personnel

Direction for 5th Mid-term plan
[FY2024-2028]

- **For further "Quality" through Regulatory Science**
  - **Consultation/review** for pharmaceuticals etc. for *the innovative products*
  - **Proper follow-up of safety measures**
  - **Emergent response system** e.g. Pandemic
- **For strategic international activities**
  - **Regulatory support/Disseminate regulatory information** to overseas companies *to develop innovative products in Japan*
- **Governance and professional personnel**

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Current Status in Japan

Current approval situation U.S./EU/Japan

	Approved	Unapproved (Total)	Unapproved products: Developing	Unapproved products: **Undeveloped**
U.S.	136	7	3	4
EU	86	57	26	31
Japan	0	**143**	57	**86**

Undeveloped items Japan

Ventures Start-up	**Orphans**	**Pediatrics**
56% (48)	47% (40)	37% (32)

※ **Of 86 items,** 14 items (16%) were not categorized as ventures, orphans or pediatrics

※ Source: Prepared by the office of Pharmaceutical Industry Research based on published information from PMDA, FDA, and EMA, and Biotoday (Technomics, Inc.), and tabulated by the Ministry of Health, Labour and Welfare.
※ 1: Of the NMEs approved in Europe and the U.S. in 2016-2020, those not approved in Japan as of the end of 2022 are counted as unapproved.
※ 2: As of March 2023, items for which no development information was available are counted as undeveloped products in Japan.
※ 3: Figures are totaled for development companies with sales of less than US$500 million within 30 years of approval in Europe and the U.S.
※ 4: Compiled as orphans for items designated as orphan drugs by the time of approval in Europe and the U.S.
※ 5: As of the end of 2022, items approved for pediatric use in Europe and the U.S. are counted as pediatrics

MHLW: Study group on pharmaceutical regulations

to strengthen drug discovery capabilities and ensure stable supplies in Japan

Start from 10 July, 2023 and ongoing now

Development	• Designation of Orphan Drugs. • Pharmaceutical reviews that contribute to the promotion of development of pediatric drugs.
Clinical Trials	• Organizing the necessity of Japanese data in the drug review • Introduction of more efficient clinical trials (ecosystem).
Post-marketing safety measures	• How to conduct post-marketing drug use results surveys. • Utilization of Real World Data in the pharmaceutical regulations.
Quality	• Approaches to drug reviews of drug manufacturing methods.
Regulatory Information	• Dissemination of information on pharmaceutical regulations in Japan to Overseas.

1st study group (10 Jul)

2nd, 3rd, 6th study group (7 Aug , 13 Sep, 13 Dec)

4th, 5th study group (13 Oct, 15 Nov)

5th study group (15 Nov)

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https://www.mhlw.go.jp/stf/shingi/other-iyaku_128701_00006.html (Japanese only)

PMDA: Regulatory Advisory Center
for Pediatric / Orphan drugs *(Tentative)*

Expected activities

1. Accelerate/expand orphan drug designation

2. To drug companies : A plan for pediatric drugs
   ⇒ *PMDA confirm the plan*

3. Accelerate the evaluation
   at “Study Group on Unapproved and Off-label Drugs of High Medical Need”

4. To companies: Subsidize the consultation fee etc.

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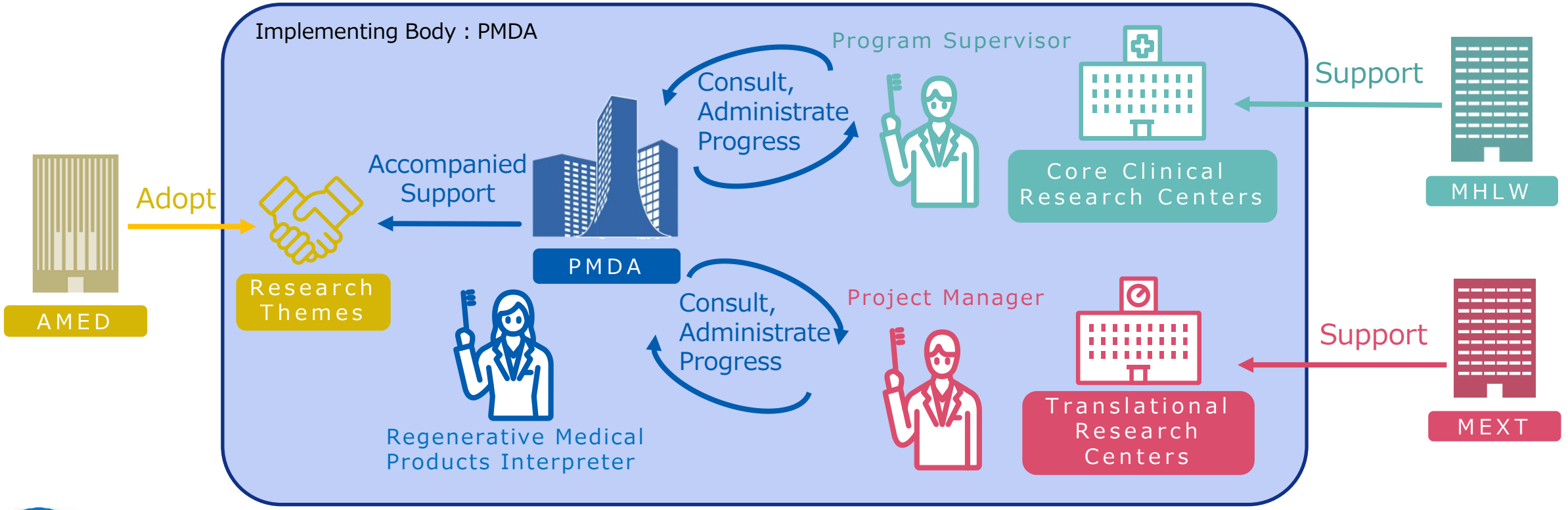
Regenerative Medical Products Interpreter

<u>Accompanied support</u> for appropriate consultation, etc.

~ from clinical research through clinical trials to product applications ~



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MHLW: Ministry of Health, Labour and Welfare
MEXT: Ministry of Education, Culture, Sports, Science and Technology

For Overseas companies
Information on Japanese Pharmaceutical Regulations

○**MHLW/PMDA systematically disseminate, in English, on their website**
○**The materials : Newly prepared/disseminated.**

You can get various supports from Japanese government if you have the willingness to develop drugs in Japan.

We, Ministry of Health, Labour and Welfare of Japan (MHLW), are looking for the companies to develop unapproved drugs in Japan!

Unapproved drugs are the ones which are approved in some European countries or US, etc. but not in Japan. In February 2010, we began accepting requests from patients including patient groups and academic societies etc. and since then has held the committee to evaluate the medical needs of such drugs.
We are looking for the companies which can develop these unapproved drugs that were evaluated as "High medical needs" in this committee but no companies in Japan which can develop them have been found. We would appreciate your cooperation so that these unapproved drugs can be developed in Japan as soon as possible and used in the medical field as well.

What kinds of support can we provide?

We will provide various supports such as subsidy, management supports, etc. when certain requirements are met for each support.

R & D
- Subsidy: Maximum up to 50% of R&D cost within budget
- Tax-deduction: 20% x [R&D cost – subsidy amount]
- R&D support: Support matching and communication between companies in joint development and coordination with regulatory authorities
- Priority consultations and Priority review

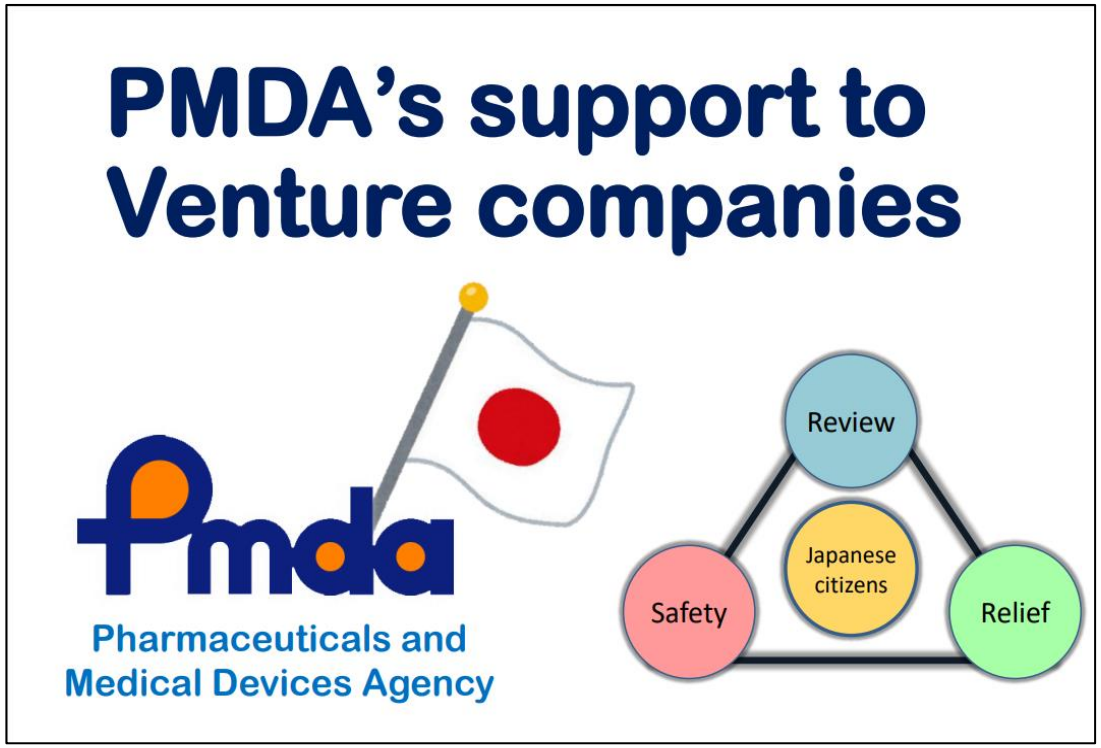
Application for approval
- Subsidy: Maximum 30 million JPY grant amount for each application
- Management support: Provide comprehensive support for each stage from R&D to practical use, and the other supports in the whole process for Startup companies

Drug price
- Incentives for drug prices

For more detailed information on the unapproved drugs we are recruiting to develop, please see this link.
Pharmaceutical Development Support Center (PDSC)
http://www.pdsc.or.jp/about/information/medicine/index.html

MHLW
For inquiries, please contact Ministry of Health, Labour and Welfare of Japan (MHLW).
Clinical Trial Promotion Office,
Research and Development Policy Division, Health Policy Bureau
Mail : Unapproved-Med@mhlw.go.jp

https://www.mhlw.go.jp/content/11120000/kousei_iyaku_shiryou_en_ol_230704.pdf



PMDA's support to Venture companies

pmda
Pharmaceuticals and Medical Devices Agency

Review
Japanese citizens
Safety
Relief

https://www.pmda.go.jp/files/000266927.pdf

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“Reliance”

Grand Design for Asian Pharmaceutical and Medical Device Regulatory Harmonization

“When a regulatory authority of one country region conducts approval reviews or inspections, they consider, attach importance to, and utilize in their regulatory activities, the outcomes of assessments made by their counterparts in other countries/regions.”

Headquarters for Healthcare Policy of Japan (20 June 2019)

Draft Good regulatory practices for regulatory oversight of medical products

“The act whereby the NRA (National Regulatory Authority) in one jurisdiction may take into account and give significant weight to assessments performed by another NRA or trusted institution, or to any other authoritative information in reaching its own decision. ”

WHO (August 2020)

Statement from Global Medicines Regulators on the Value of Regulatory Reliance

“Regulatory reliance, which is a mechanism to strengthen regulatory capacity, to improve health systems nationally and internationally, to increase the availability of medicines, to save financial resources and to use human resources more strategically.“

ICMRA (27 November 2020)

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Momentum: “reliance” for worldwide!

PMDA-ATC

Asia Training Center for Pharmaceuticals and Medical Devices Regulatory Affairs

Capacity building/human resource development for Asian regulators

Action Policy of PMDA-ATC

Universal health coverage through regulatory harmonization in Asia

PMDA-ATC

Asian countries

Site visit/ Lectures /Case studies /Practical trainings

Tailored trainings to needs

Japan

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APEC Asia-Pacific Economic Cooperation

Invites Asian regulators

Input Japanese knowledge /experiences

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The Commemorative Summit for the 50th Year of ASEAN-Japan Friendship and Cooperation

2. Partners for Co-creation of Economy and Society of the Future

2.8 Health

2.8.6 Promote cooperation on expansion of access to quality health services, pharmaceuticals and medical devices for non-communicable diseases including training in regulating pharmaceuticals and medical devices through platforms such as the Asia Training Center for Pharmaceuticals and Medical Devices Regulatory Affairs (PMDA-ATC);

(Photos: Cabinet Public Affairs Office)

Excerpt from the "Implementation Plan of the Joint Vision Statement on ASEAN-Japan Friendship and Cooperation"

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PMDA-ATC Seminars
(Open to all regulators)

Trainings: FY 2023 (April 2023 to March 2024)

	Contents	Period (days)	Location
1	Pediatric Review*1	4	Tokyo (PMDA)
2	Quality Control (Herbal Medicine)	3	Toyama
3	Pharmaceuticals Review	3	Online
4	Medical Devices*2	3	Online
5	Medical Devices	3	Tokyo (PMDA)
6	Multi-Regional Clinical Trial (MRCT) *2, 3	4	Tokyo (PMDA)
7	Good Manufacturing Practice (GMP)	3	Online
8	Pharmacovigilance*2	4	Online

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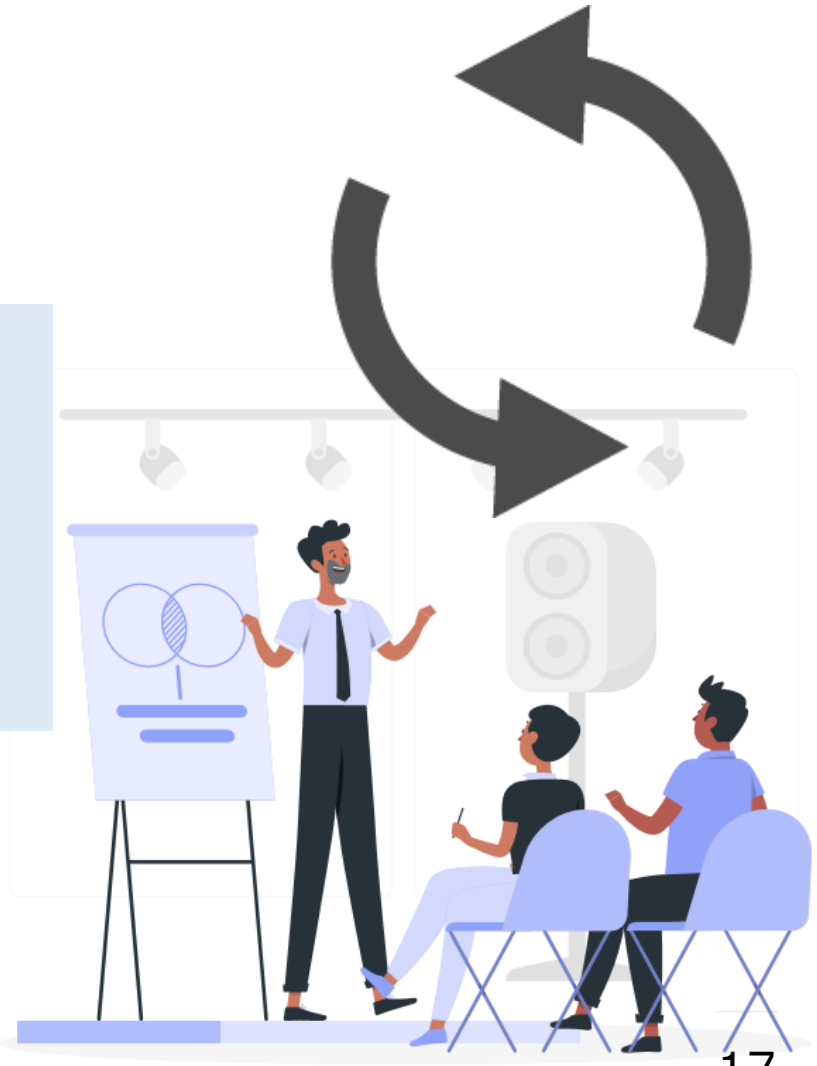
*1 Joint Seminar with U.S.FDA
*2 APEC CoE Workshop
*3 Collaboration with National Cancer Center Japan

Updating the PMDA-ATC Training Seminars

PMDA-ATC

More well-organized, thoughtful, and practical trainings

- **Questionnaire**: From ATC Seminar Participants
- **Feedback**: For refined seminar



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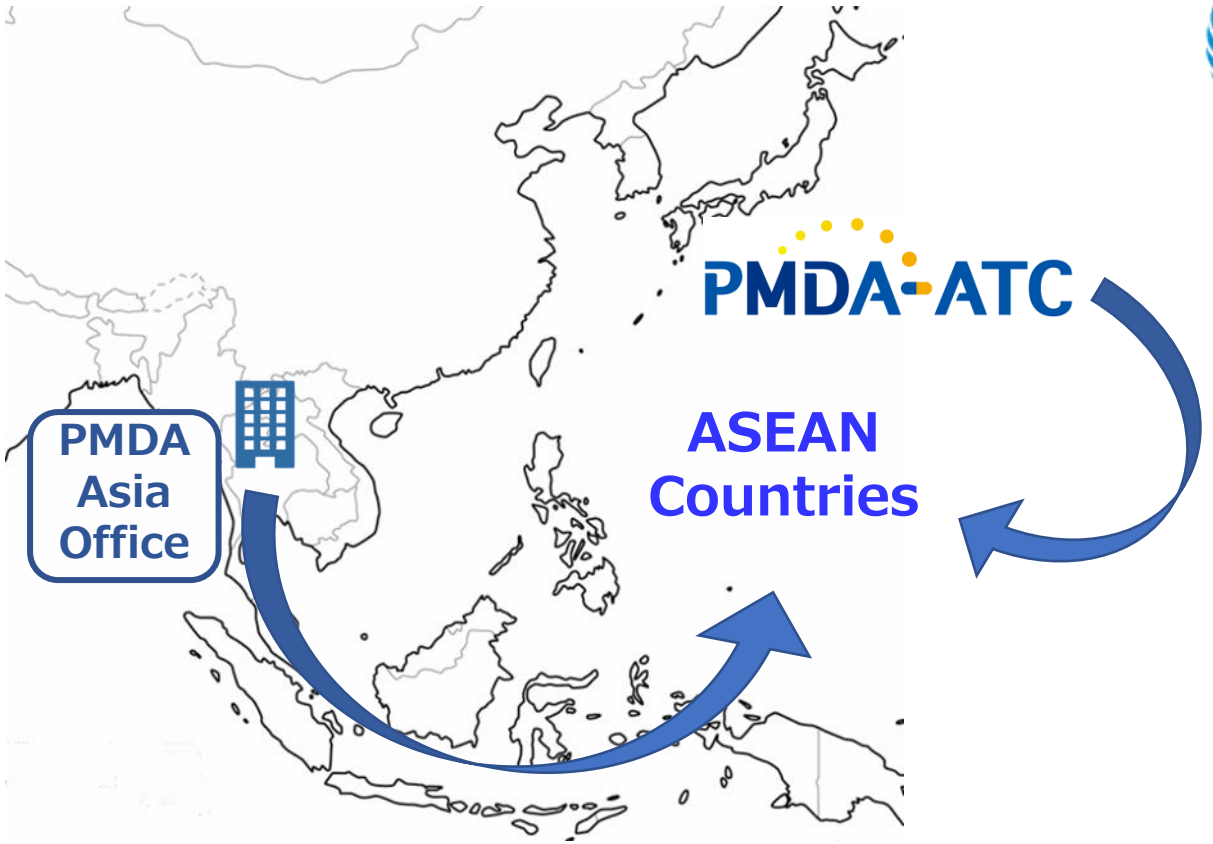
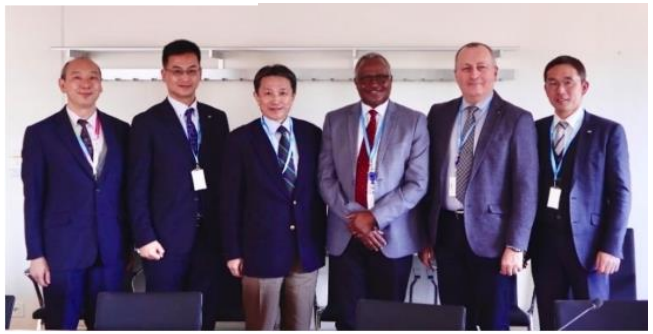
Cooperation between WHO and PMDA on training for regulatory authorities



World Health Organization

PMDA-ATC



- Systematizing:
  Collaboration on trainings/seminars by PMDA/WHO through facilitators

- Effective capacity building/training in ASEAN countries.

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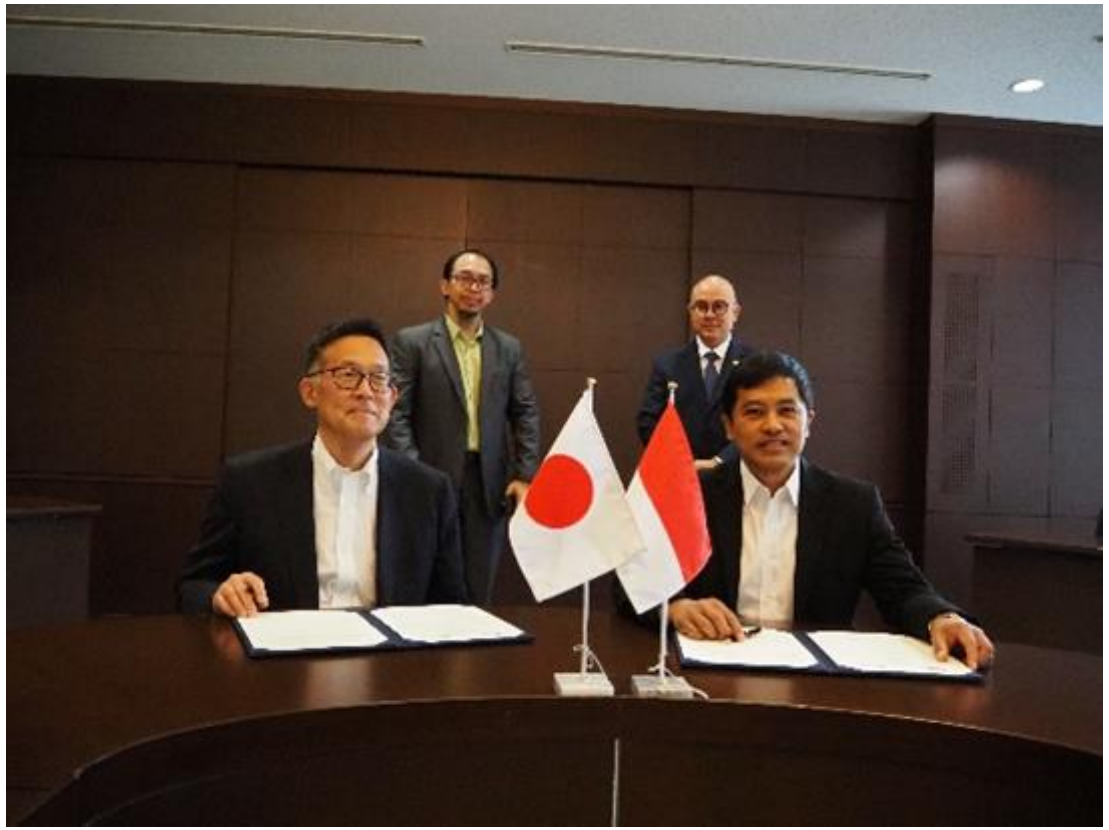
Long-term Training Program

Letter of Intent on a long-term training program

signed by The Ministry of Health of the Republic of Indonesia (MoH) & PMDA

Training for medical device regulation

under the framework of the PMDA-ATC



Signing Ceremony of the Letter of Intent (LOI) on Long-term Training Program on 5th July, 2023

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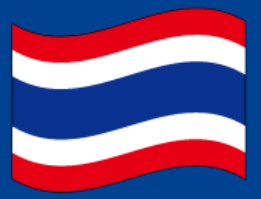
PMDA Updates August 2023 https://www.pmda.go.jp/files/000263976.pdf

PMDA Asia Office
(Bangkok, Thailand)



Objective

Contribute to innovative products access with on-site communication

through

- *Cooperation with ASEAN regulators*
- *Regulatory harmonisation with Asian countries*
- *Collaboration: Clinical research network*

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International Collaboration and Reliance

Significantly important than ever before

- Globalisation of supply chain
- Emergence of new technologies
- Limited human resources
- Response and Preparedness for pandemic (COVID-19 and the Next), etc…

Fast/Stable access
through reliance enhancement

ex. Contribute: ASEAN Joint Assessment scheme (by WHO/PMDA collaboration)

https://www.pmda.go.jp/english/int-activities/0010.html

World Health Organization

PMDA-ATC

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Thank you for your attention

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