Dr Lembit Rägo
Secretary-General
Council for International Organizations of Medical Sciences (CIOMS)

Introduction

CIOMS – Introduction (https://cioms.ch/)

Council for
International
Organizations of
Medical
Sciences

Founded in 1949 by WHO and UNESCO

In official relations with WHO

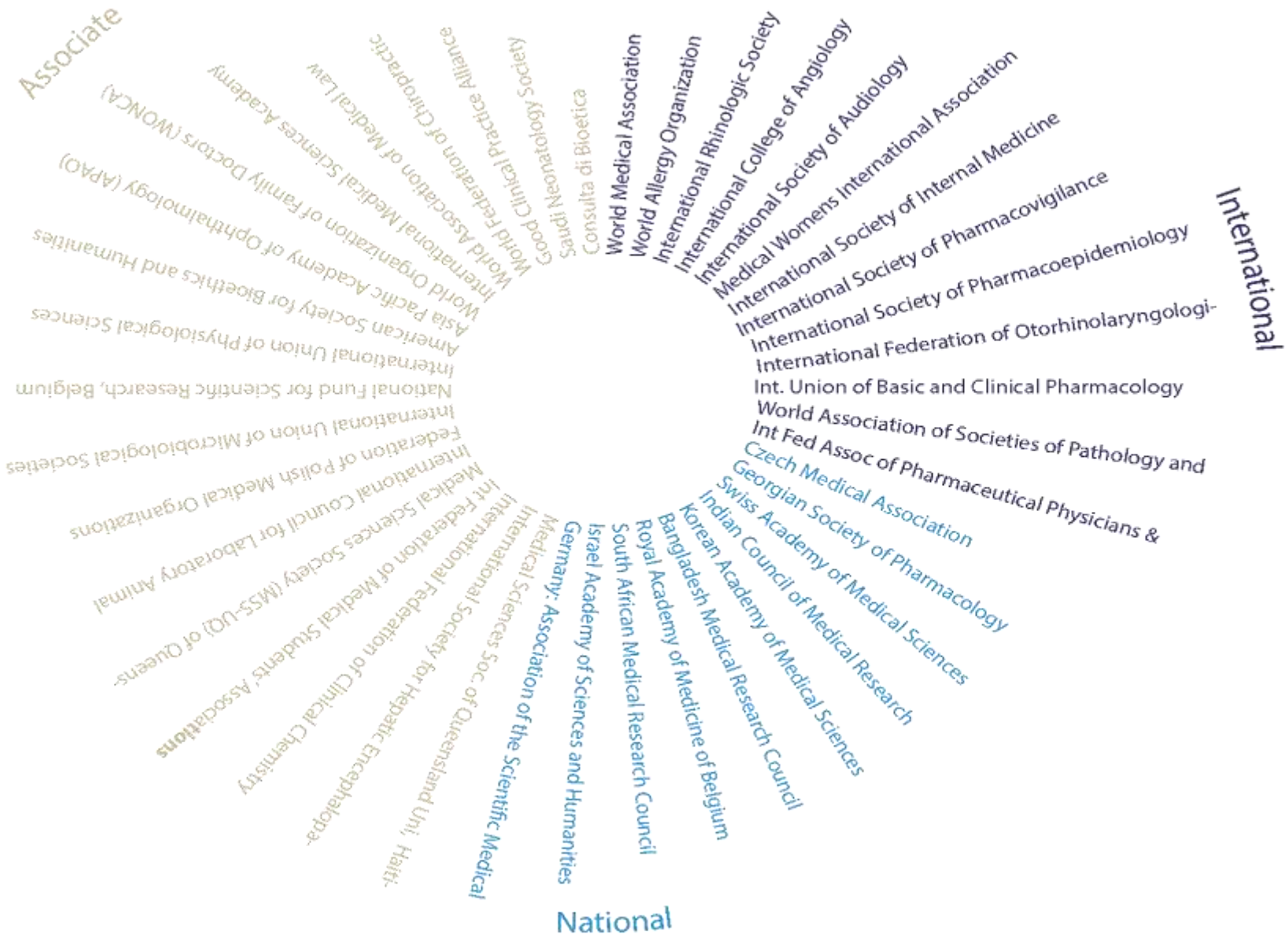
UNESCO associated partner

ICH Observer since 2016

Mission Statement

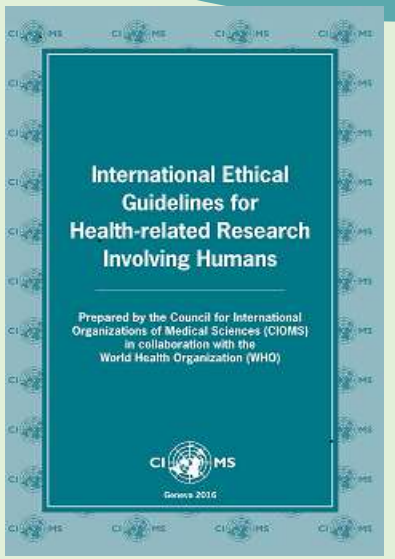
CIOMS mission is to advance public health through guidance on health research including ethics, medical product development and safety

CIOMS Member organizations



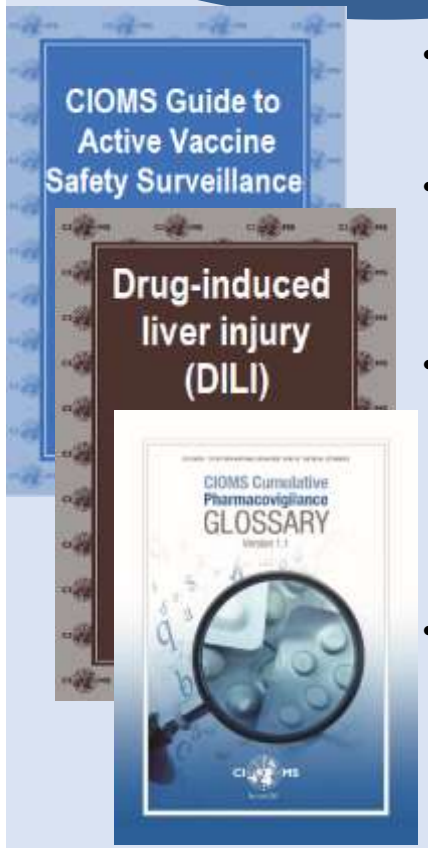
Major areas of work

Bioethics



- Since 1967; 1st CIOMS Round Table Conference 'Biomedical Science and the dilemma of Human Experimentation'
- Issued significant guidelines
  - Latest revision 2016
  - Focus on 'low -and middle- income countries'
  - Available in 10 languages, e.g. Chinese, Spanish, Japanese, Russian

Pharmaco-vigilance



- 1986: first PV Working Group
- 13 more working group reports to date
- Several ICH Guidelines are based on results of CIOMS Working Groups
- Cumulative Glossary 2021

Product development

- Since 1977 CIOMS Round Table Conference, 'Trends and Prospects in Drug Research and Development' ….
- 2021: Clinical Research in Resource-Limited Settings, CIOMS Working Group report
- 2022: Glossary of ICH terms and definitions

CIOMS Working Groups in 2022

Working Group topic and starting date
1. MedDRA Labelling Groupings. *Started April 2019*
2. Benefit-Risk Balance for Medicinal Products. CIOMS WG XII. *Started September 2019*
3. Real-World Data and Real-World Evidence in Regulatory Decision-Making. CIOMS WG XIII. *Started March 2020*
4. Severe Cutaneous Adverse Reactions (SCARs). *Started February 2021*
5. Recommended Standards of Education and Training for Health Professionals Participating in Medicines Development. *Started April 2021*
6. Good Governance for Research Institutions. Started July 2021
7. Artificial Intelligence in Pharmacovigilance. CIOMS WG XIV. *Started May 2022*

CIOMS Newsletter gives updates - https://cioms.ch

This webinar is about introducing the new CIOMS report

How we do it?

Two speakers

Elisabeth Oehrlein

Manal Younus

Panel discussion moderated by

Peter Pitts

Thank you!

And now over to our speakers
and moderator to continue

Report content

- Ethical considerations for patient involvement
- Executive summary
- Chapter 1: Introduction
- Chapter 2: Landscape
- Chapter 3: Guiding principles
- Chapter 4: Advancing treatments
- Chapter 5: Use of real-world data and evidence
- Chapter 6: Product labeling
- Chapter 7: Rapid safety communication
- Chapter 8: Additional risk minimization
- Chapter 9: Clinical practice guideline
- Chapter 10: Low- and middle-income countries
- Chapter 11: Pandemic considerations

Appendices:

1. Glossary
2. Case studies
3. CIOMS WG XI statement
4. CIOMS WG membership and meetings
5. List of commentators

Acknowledgements

International organisations, academia and other stakeholders

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Younus, Manal*	International Society of Pharmacovigilance (ISoP)

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Houÿez, François	EURORDIS-Rare Diseases Europe
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Sakushima, Ken	Pharmaceuticals and Medical Devices Agency (PMDA), Japan
Sato, Daisaku**	Pharmaceuticals and Medical Devices Agency (PMDA), Japan, moved to Ministry of Health, Labour & Welfare, Japan
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Industry

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Kugener, Veronique** ..... Takeda
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Noel, Rebecca ..... Eli Lilly
O'Hare, Mary* ..... Roche
Patel, Ravi ..... United Therapeutics
Pawar, Ameet ..... Takeda
Peppers, Laura* ..... Takeda
Purevjal, Indra ..... Takeda
Renz, Cheryl ..... Retired from AbbVie
Richardson, Michael* ..... Bristol Myers Squibb
Russo, Leo ..... Pfizer
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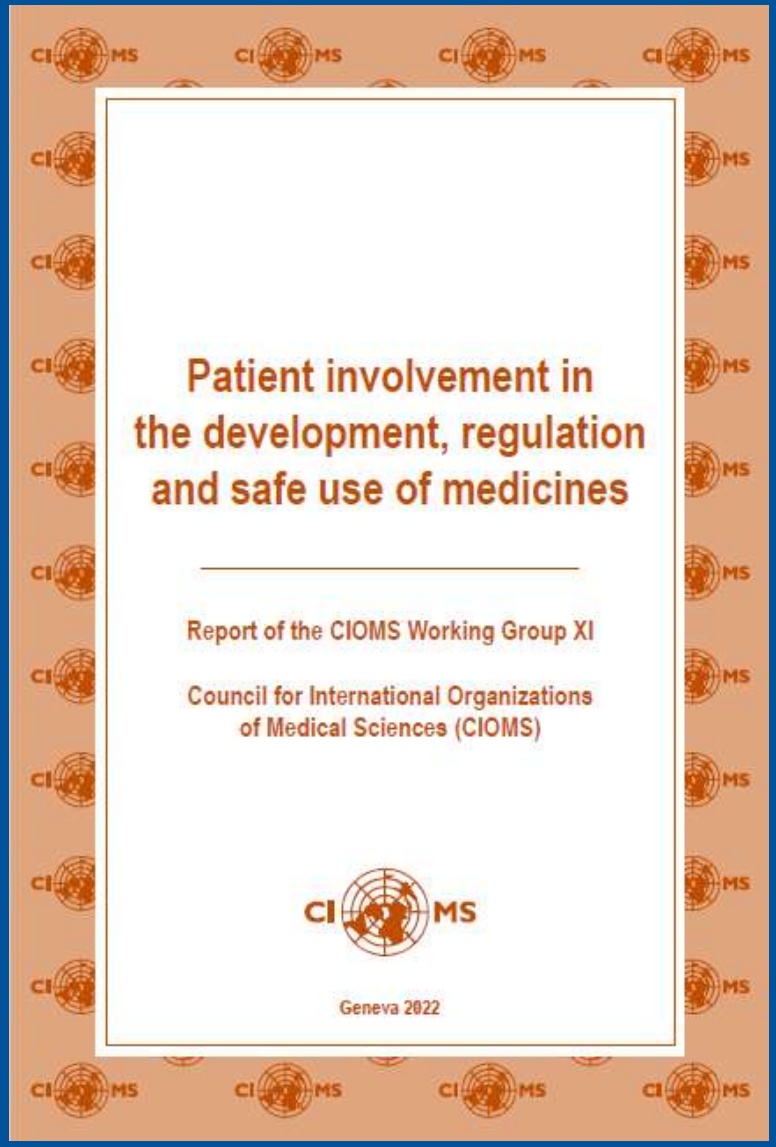
Smith, Meredith ..... Alexion
Stürchler, Christine ..... Novartis
Webster, Peggy** ..... Takeda
Wilkins, Jamie* ..... Pfizer

Chapter 1: Introduction
Chapter 2: Landscape
Chapter 3: Guiding principles
Chapter 4: Advancing treatments
Chapter 5: Use of real-world data and evidence



Terminology

- **Patient community**: broadly encompasses
  - Individual patients
  - Family caregivers
  - Organizations that represent them
- **Patient**: A person who has, or had, a health condition whether or not they currently receive therapy to prevent or treat it.

Adapted from: The National Health Council Rubric to Capture the Patient Voice: A Guide to Incorporating the Patient Voice into the Health Ecosystem. June 2019. Washington, DC and the Food and Drug Administration's Patient-Focused Drug Development Glossary.

Terminology

- **Patient-focused drug development (PFDD):** A systematic approach to capture patients' experiences, perspectives, needs and priorities, and to incorporate them meaningfully into the development and evaluation of a medicinal product throughout its lifecycle.
- **Patient engagement (synonym: Patient involvement):** The active, non-tokenistic and collaborative interaction between patients, the patient community and other stakeholders, where decision making is guided by patients' contributions as partners, recognising their unique experiences, values and expertise.

Adapted from: U.S. Food and Drug Administration. Patient-Focused Drug Development Glossary.
Adapted from: Harrington RL, Hanna ML, Oehrlein EM, Camp R, Wheeler R, Cooblall C, *et al.* Defining Patient Engagement in Research: Results of a Systematic Review and Analysis: Report of the ISPOR Patient-Centered Special Interest Group. Value Health. 2020 Jun;23(6):677–688. doi: 10.1016/j.jval.2020.01.019

Chapter 1: Introduction

1.1	**Terminology**
	Opportunities to incorporate the patient's perspective
1.2	**Increasing engagement and incorporating the patient's perspective**

- Solid blue arrow depicts how a medicine may progresses to marketing authorization

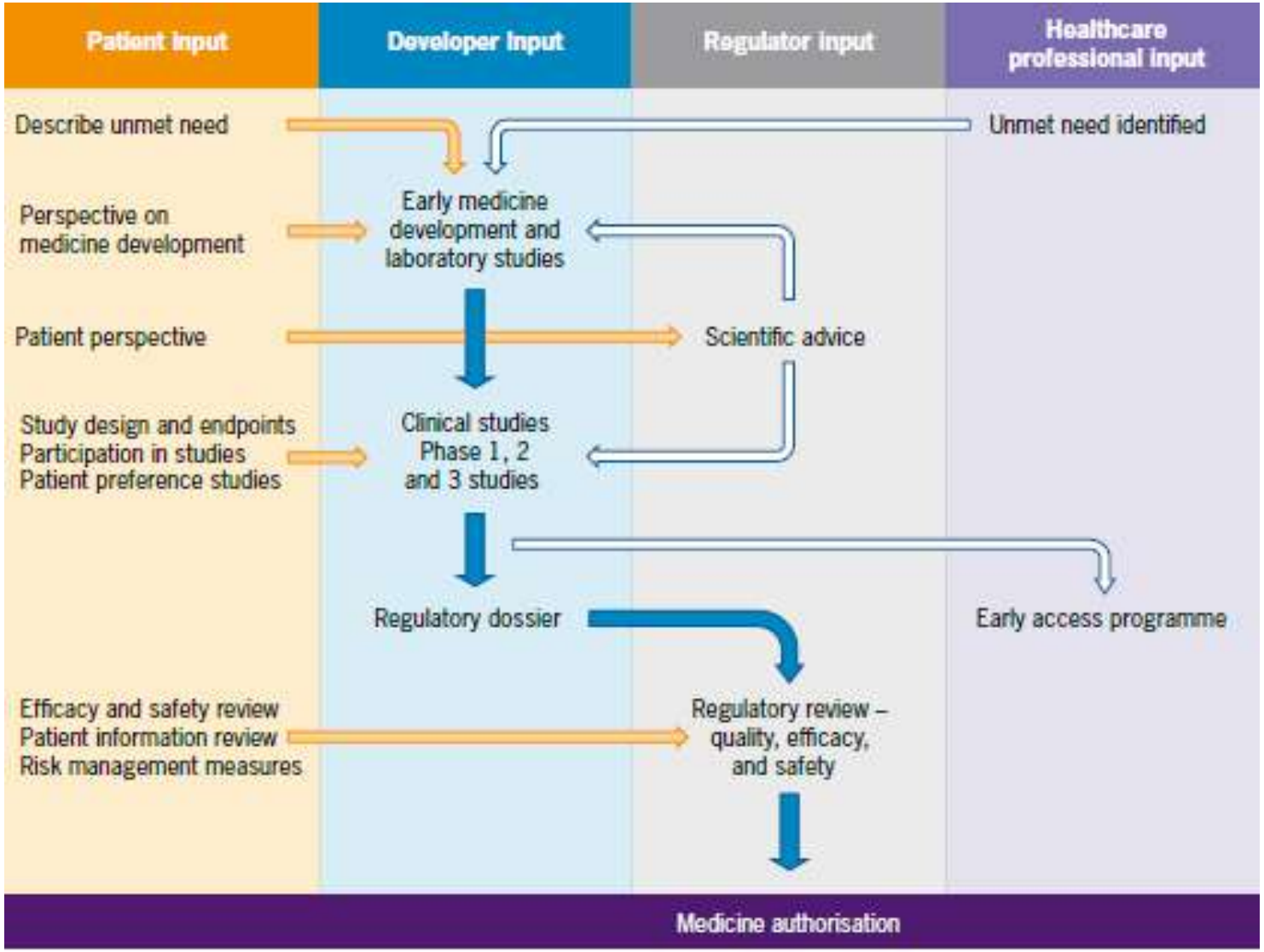


Figure 1a: Patient involvement during a medicine lifecycle: ***pre-authorisation***

Source: CIOMS Working Group WG XI

Chapter 1: Introduction

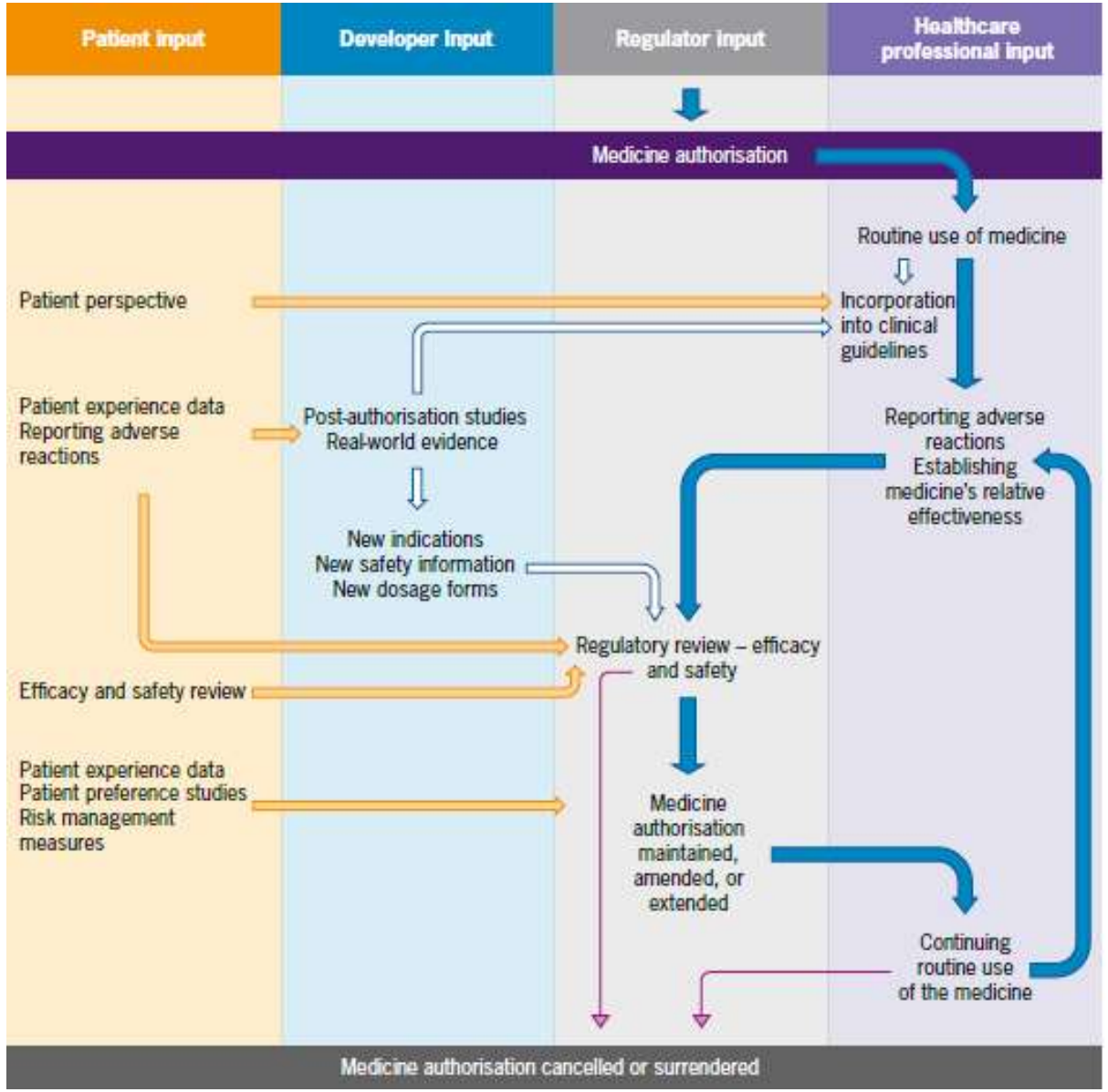


Figure 1b: Patient involvement during a medicine lifecycle: ***post authorisation***

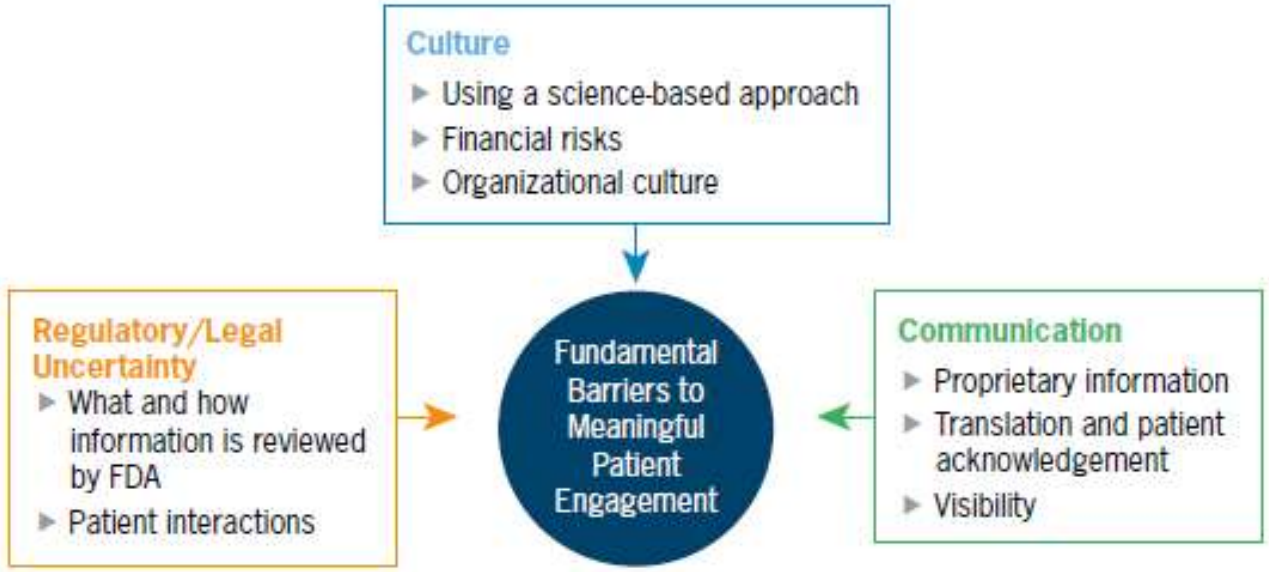
Patient involvement throughout the post-authorisation phase is essential:

- Understanding how well the medicine addresses patients' needs
- Identifying any new concerns and developing strategies to minimise them

Source: CIOMS Working Group WG XI

Chapter 2: Landscape

Barriers to meaningful engagement in medicine development identified at National Health Council/Genetic Alliance Dialogue (2015)



Source: National Health Council/Genetic Alliance (Figure reproduced with permission)

Chapter 2: Landscape: key points

1. Patient advocates, especially members of the HIV/AIDS and rare disease communities, advanced the role of patients in the development and regulation of treatments.

2. Patients, pharmaceutical companies and medicine regulators have collaborated to overcome real and perceived regulatory, cultural and communication barriers to patient engagement in medicines development.

3. Case examples of patient involvement in the development, regulation and use of medicines demonstrate considerable benefit to all parties: a win-win situation.

4. The cultural shift to greater involvement of patients needs to continue by deepening involvement of patients in areas such as:
    a. Identifying patient-centered outcomes (also called patient-relevant outcomes);
    b. Participating in regulatory review;
    c. Contributing to constructing, reviewing and disseminating medicines information;
    d. Monitoring medicines safety by making direct contribution to reporting and assessing side effects.

Chapter 3: Guiding principles

3.1		**The patient voice is vital**
	3.1.1	Clarifying goals that are important to patients
	3.1.2	Who should engage and when
3.2		**Patients' expert knowledge and credibility development**
3.3		**Reimbursement of expenses and compensation for patients' time and contribution**
	3.3.1	Reimbursing expenses for participation
	3.3.2	Compensation for patient's time and expertise
3.4		**Training of stakeholders for patient engagement activities**
	3.4.1	Training and education of those who engage patients
	3.4.2	Training and education of patients for patient engagement activities
3.5		**The independence of patient**
	3.5.1	Patients' independence in patient engagement activities
	3.5.2	Patient engagement must not result in promotion or endorsement of a medicine
	3.5.3	Funding of patient organization
	3.5.4	Optimizing patient organization input
3.6		**Transparency, open communication and agreements**
	3.6.1	Open and honest communication
	3.6.2	Disclosure of conflict of interest
	3.6.3	Contract and agreement need to be brief and clear
	3.6.4	Transparency of stakeholder relationships while protecting privacy

Chapter 3: Guiding principles

3.4. Training of stakeholders for patient engagement activities

Guiding principle. Consider training of all stakeholders during the planning for patient engagement activities.

3.4.1. Training and education of those who engage patients

Effectively engaging with patients requires specific knowledge, skills and experience. It should not be assumed that an organisation is ready to engage patients without first assessing current capabilities. Organisational training and education are key for building these capabilities.

In addition to relevant regulatory, legal, and healthcare compliance requirements, and specific patient engagement approaches and methods (*e.g.* patient advisory boards), other topics for organisational training and education include:[10,15]

Skills

Awareness

- Understanding the nature of patient representatives, their organisations and how they operate;
- Where to find patient representatives or organisations and how to determine who to work with;
- Case studies and testimonials of the importance and value of patient involvement beyond trial participation in the medicine development lifecycle.

Technical skills

- Evaluation tools and metrics to assess the effectiveness and impact of patient engagement;
- Interpreting, integrating, handling and protecting data generated from patient engagement into medicine development and regulatory activities;
- Communication skills to convey medical and technical concepts and transferring knowledge effectively to partners who do not have technical or scientific backgrounds.

Other skills

- Dispelling preconceived notions about patient representatives and organisations (their abilities, their knowledge of medicine development, and their motives or intentions);
- Listening skills to discern meaning from spoken and unspoken communications from a person or group of people;
- Cultural sensitivity to understand differences across cultures and subtle differences among social groups, patients, and those underrepresented or discriminated against.

Chapter 3: Guiding principles

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Resources

- DIA Considerations Guide for Implementing Patient-Centric Initiatives in Health Care Product Development – link
- IMI-PARADIGM, D4.1 Recommendations on the required capabilities for patient engagement – link
- EMA Training Strategy for patients and consumers involved in EMA activities – link
- EUPATI. EUPATI Fundamentals - Training for Professionals. 2019 – link
- EUPATI. EUPATI Training Course. 2018. Retrieved from EUPATI European Patients' Academy – link
- EURODIS. EURORDIS Open Academy. Retrieved from EURODIS Rare Diseases Europe. 2019 – link
- National Health Council. Center of Educational Excellence – link
- PFMD Book of Good Practices – link
- PFMD. Patient engagement industry training. 2019 – link
- Warner K, See W, Haerry D, Klingmann I, Hunter A, May M. EUPATI guidance for patient involvement in medicines research and development (R&D); guidance for pharmaceutical industry-led medicines R&D. Front Med. 2018;5:270 – link

Chapter 3: Guiding principles: key points

1. The patient voice offers a valuable perspective throughout the development of a medicine. It should be fully incorporated into the decision-making process.
2. Patients have expert knowledge and understanding of their diseases and conditions. This means they have equal credibility as those who are scientific and medical experts.
3. Reimbursement of expenses and compensation for patients' time and contribution should be considered.
4. Consider training of all stakeholders during the planning for patient engagement activities
5. Every effort should be made to maintain patients' independence
6. Balanced information, transparency and open communication are key. Written agreements should be easy to understand and complete

Chapter 4: Advancing treatments

Chapter 4: Advancing treatments

Example opportunities for patient input to guide medicines development:

1. Evaluation of the impact of a medicine (choice of the patient-relevant outcome measure or clinical assessment outcome).
2. Improving the conduct of a clinical trial, by discussing:
    a. how to reduce the burden of procedures in a trial;
    b. how patients can be helped to sign up to take part in a study;
    c. increasing the diversity of participating patients;
    d. ways to encourage patients to continue to stay in a study until the trial ends;
    e. any substantial amendment to the protocol.

Chapter 4. Table 3. Stakeholder collaboration examples on introducing, improving, and using medicines

Ongoing →

Stage:	Unmet need	Early development	Clinical development	Regulatory review	Healthcare delivery Safety monitoring	Health & data communication
Patients	• Form patient organisations • Produce information for patients about their disease • Conduct / contribute to early research • Create patient registries • Create biosample banks • Develop research priority setting partnerships, *e.g.* James Lind Alliance (see section 5.3.7)	• Establish research priorities • Describe living with disease • Describe standard of care – may not be treatments available (likely to be some variability) • Describe being treated • Describe needs, goals and wants	• Develop patient-relevant outcomes • Contribute to protocol design • Contribute to benefit-risk profile • Co-create / review research plans • ASTERIX (Advances in Small Trials dEsign for Regulatory Innovation and eXcellence) • Co-create / review information for patients • FDA MyStudies App	• Contribute to dossiers / reviews • Members of scientific committees • EMA involvement • FDA collaborative process • User-test patient leaflets and some risk management materials	• Learn about treatments • Contact developers about promising products for compassionate use • Talk about treatments and goals with HCP • Tell HCP / sponsor / regulator about side effects • Engage conversations with developers following a safety signal once the product is on the market. This may be the first dialog between patients and drug developers.	• Co-create / review non-promotional information • Co-create / contribute (to) good information guidance
Healthcare professionals	• Establish clinical guidelines • Characterise disease • Develop natural history studies	• Talk with / listen to patients about their needs, goals, and wants	• Inform patients about clinical trials and ensure they are making an informed choice • Discuss with patients their interest and eligibility for clinical trials • Support patients throughout the trial and give regular feedback • Talk about standard treatment	• Give input on current treatment regimens	• Learn about safe and appropriate use of product • Report side effects promptly • Engage with patients to establish treatment guidelines	• Co-create / review / distribute non-promotional materials
Developers	• Joint research priority partnership, *e.g.* The James Lind Alliance (see section 5.3.7)	• Discuss with patients their needs, goals, and wants • PFMD	• Co-create with patients or request patient review of research plans; incorporate needed changes • Co-create with patients or request patient review of information for patients; incorporate needed changes • Developers contact patient organisations to recruit for clinical trials (should not be the first interaction with patients) • Provide clinical trial feedback to patients (make accessible)	• Include patient input in dossiers • Propose patient-oriented labelling	• Monitor safety and effectiveness of treatments in patient-friendly ways • Involve patients in risk minimisation planning and activities; see also CIOMS Working Group IX report	• Co-create non-promotional information per guidance
Regulators	• Early dialogue/hearing with patients and patient groups (*e.g.* EMA's Innovation Task Force and FDA's Patient Listening Sessions) • Involvement in orphan designation • Involvement in scientific advice	• Invite / attend public discussions of patients' diseases, treatments, needs, goals, and wants • FDA CDER PFDD • EMA multistakeholder workshops • Talk with sponsors and patients about development plans	• Co-create with patients and provide guidance on including patients' input in treatment development • FDA CDER PFDD • EMA patients & consumers • EMA scientific advice • Talk with sponsors and patients about development plans and risk minimisation • Include patients as members of scientific committees, *e.g.* EMA paediatric committee PDCO, committee for orphan medicine	• Include patient input in review of dossiers • EMA scientific committees review process • Include user-testing for patient leaflets and relevant risk management materials	• Monitor safety and effectiveness of treatments in patient-friendly ways • EMA PV stakeholder forum • FDA RWE Framework • Hold public hearings for input	• Co-create / provide guidance on including patients' input in non-promotional information • EMA review of documents

Source: CIOMS Working Group WG XI

Chapter 4: Advancing treatments

4.6. How to engage

Sponsors may partner with patients in several ways, including conducting in-depth interviews, focus groups, participation on advisory boards, trial simulations, user testing of study devices, review of educational materials, or sponsors may attend community advisory boards sponsored by patient groups.[19,26]

Interactions with individual patients and group meetings may be conducted in-person or through videoconferencing, phone calls, social media or online patient surveys.

User testing is used routinely in the EU to make sure information is fit for purpose (see also section 2.2.7, section 6.6 and section 8.3.4). Once proposed improvements have been implemented, patient leaflets will have improved legibility, clarity and ease of use. User testing with 'real' patients – members of the public who are not necessarily skilled readers – highlights readability problems in a document.[27]

Some patient organisations can help to identify patients to provide feedback based on the input being sought (*e.g.* review of informed consent form, feedback on elements of protocol, patient material development). For example, EUPATI-trained patients can provide input in a number of areas.

This field is evolving fast and here are some examples from EMA's Innovative Medicines Initiative (IMI) projects – the multiple stakeholder projects to improve health by speeding up the development of, and patient access to, innovative medicines, particularly in areas where there is an unmet medical or social need:

- PARADIGM delivered 'an inventive and workable sustainability roadmap to optimise patient engagement in key decision-making points across medicines'.[28]
- PREFER delivered expert and evidence-based guidance on when and how to design and conduct a patient preference study.[36] (See the following section 4.7.)
- PROTECT addressed knowledge gaps and data needs that represented barriers to product development and continuous benefit-risk monitoring of medicinal products during their lifecycle.[29]

Chapter 4: Figure 3. 'Map My Experience' patient experience mapping tool

Example method to gather information about patient experiences and unmet needs

Source: Oehrlein EM, Schoch S, et al. Patient Experience Mapping Toolbox. National Health Council; 2021. Available from: https://nationalhealthcouncil.org/resources/patient-experience-map1 (used with permission)

Chapter 4: Advancing treatments

4.7 Patient engagement in patient preference studies:

Strategies to empower patients effectively

- providing clear, concise descriptions of the patients' or patient partners' roles
- offering flexibility around meeting times and assistance with transportation
- providing opportunities to participate remotely (e.g., by video conferencing);

IMI-PREFER, a 5-year, multi-stakeholder initiative to provide evidence-based recommendations on how and when Patient Preference Studies (PPS) should be performed to inform medical decision making, has proposed the following principles for interacting with patients in the context of a PPS.[33]

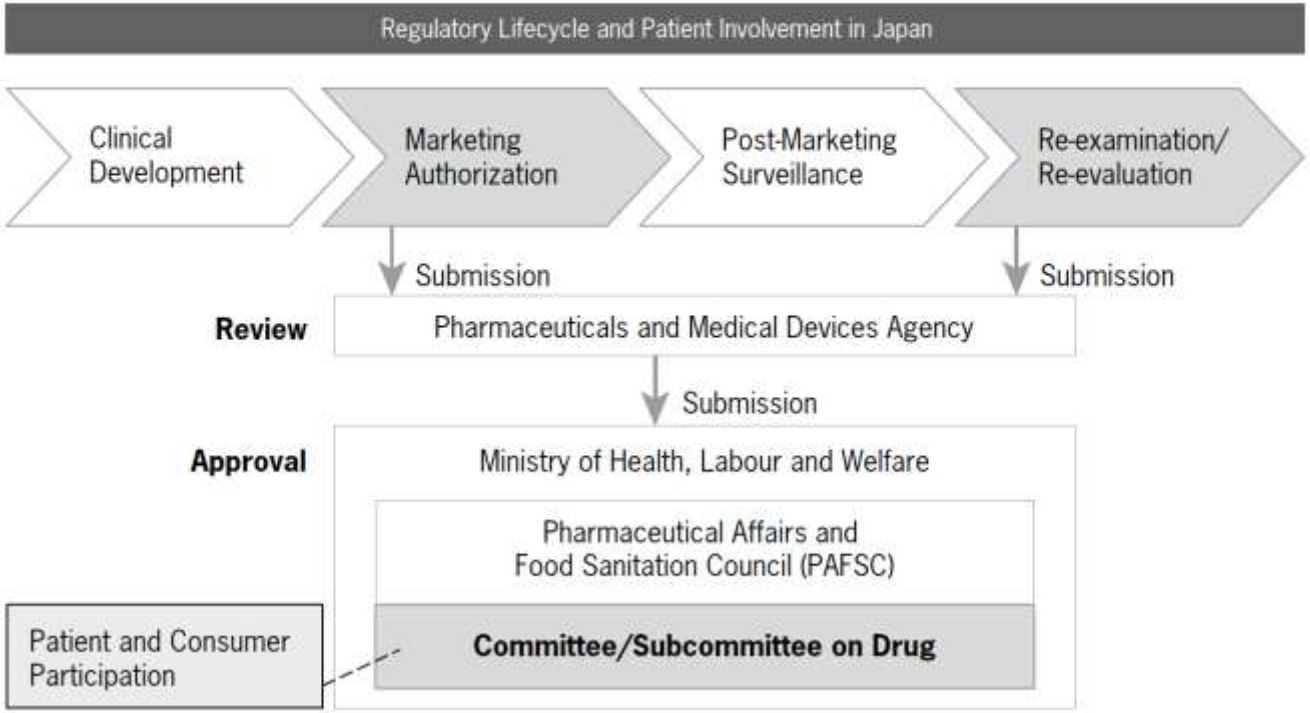
1. Use easy to understand, non-technical language, and include glossaries of technical terms where required.
2. Clearly and concisely describe the roles of patient research partners.
3. Undertake outreach work to involve patient research partners in community settings.
4. Enable flexibility around meeting times, including out-of-office hours.
5. Use easily accessible meeting venues (*e.g.* lifts/ramps, locations)
6. Provide opportunities for patient research partners to contribute remotely (*e.g.* via email, teleconferences, video meetings).
7. Ensure meetings are structured to accommodate the needs of patient research partners (*e.g.* frequent breaks, refreshments, lay summaries of presentations/documents, care givers can attend)
8. Reimburse any expenses and payments for time spent.
9. Provide recaps at regular intervals of the study background and objectives, progress updates, and the impact of the patient research partner activities.
10. Allow sufficient time for the completion of involvement activities.
11. Ensure there is no requirement for patient research partners to sign or review lengthy and/or complex documents or legal agreements.
12. Ensure patient research partners have the requisite skills and knowledge to support meaningful involvement (*e.g.* to enable patients to contribute to aspects of data analysis or study conduct, assertiveness skills to support participation in management meetings). This may require specific training or provision of information or support.
13. Provide training for study sponsors so that they can effectively involve members of the public (*e.g.* communication skills, needs awareness, outreach training).

4.8 Patient engagement in regulatory review

- Patients' participation in regulatory activities can be categorised as follows:
  - Patients representing 'patient community' interest e.g., through nomination to a regulatory authority management board or a scientific committee;
  - Patients, representing their organisations, who participate in a public consultation on specific guidelines or act as advocates on a specific disease condition;
  - Patients providing individual expertise on their own disease, for example, during the evaluation of a marketing authorisation application;
  - Patients commenting as a member of the general public, for example, on an issue posted for public consultation.

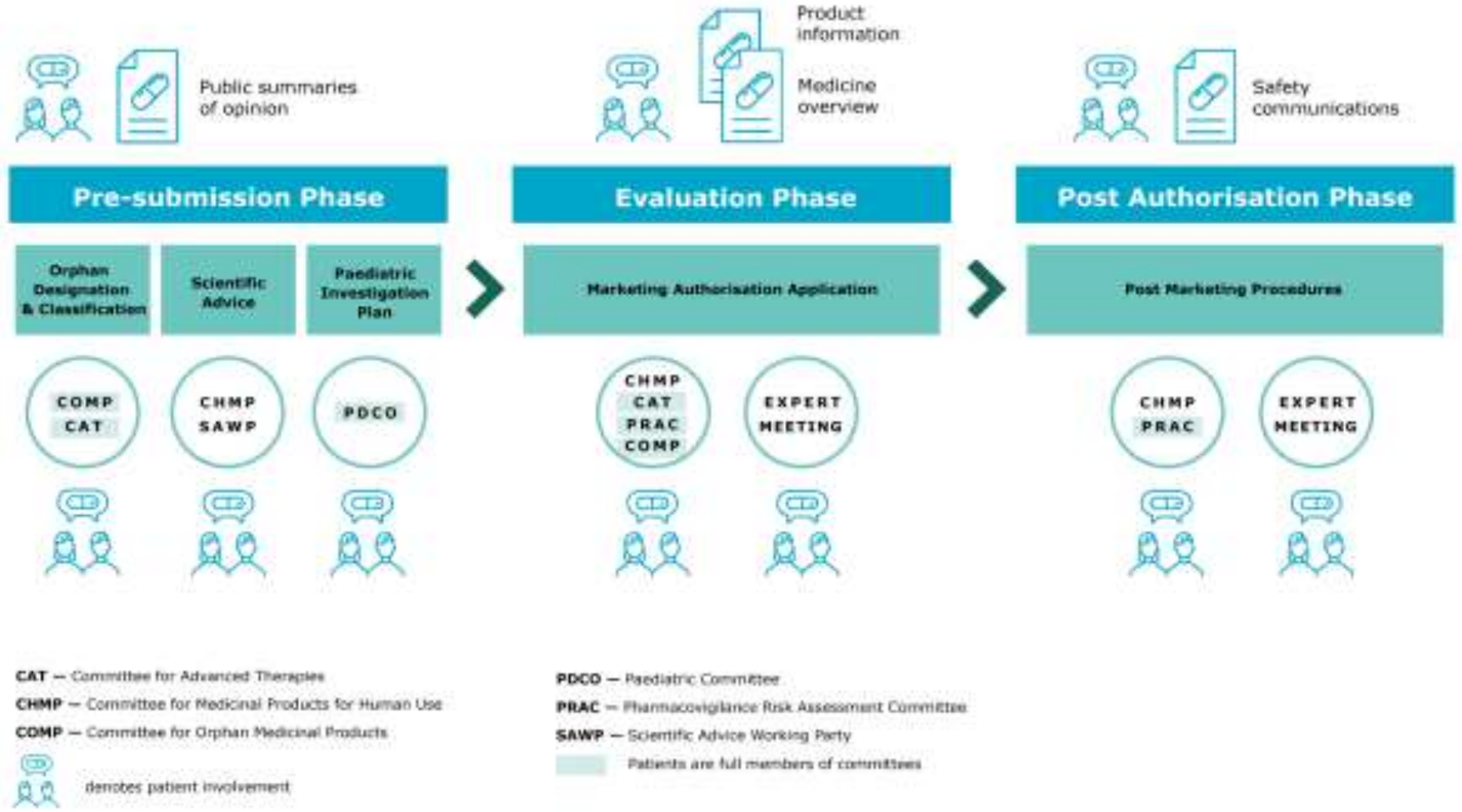
Figure 5: Patient involvement in the medicines lifecycle at the Pharmaceuticals and Medical Devices Agency, Japan

Source: Modified by Pharmaceuticals and Medical Devices Agency (PMDA), Japan, from chart entitled "Flow of Examination for the Approval of a New Pharmaceutical", Health and Medical Services, p. 93. (PDF accessed 17 February 2022)[41]



Chapter 4. Figure 4. Patient involvement in the medicine lifecycle at European Medicines Agency

Touchpoints for patient engagement at EMA during a medicine's lifecycle



Source: Kindly provided by the European Medicines Agency

Chapter 4: Advancing treatments: key points

1. Many stakeholders are involved in discovering treatments, developing them through the product lifecycle, and promoting their safe use.
2. Stakeholders include patients themselves, along with healthcare professionals, sponsors (academics, funders, and biotechnology developers), regulators, and payers.
3. Patient participation is needed in planning, testing, reviewing, approving, and monitoring treatments throughout the lifecycle of medicines.
4. Improving treatment development and delivery depends on transparent and evidence-based communications among all stakeholders

Chapter 5. Use of real-world data and evidence

Chapter 5. Use of real-world data and evidence

5.3.3. Data ownership or control

While patients are largely in favour of sharing their data, they still wish to keep control of the data-sharing process. Respondents to the EURORDIS survey were overwhelmingly in favour of having the strictest control on their data.

The European Patients' Forum, too, has expressed this view. It states in its 2020 response to the European Commission's data strategy:

> *Patients must be in control of their data. They should be able to freely access it, decide who to share it with, and on what conditions… It should be possible for those individuals who wish to do so, to give wider access to the data held about them (e.g. through so-called data altruism or data donation), as long as the implications of doing so are fully transparent and clear. Patients want to know and have some control over what purposes their data is used for and track its use when possible, and they often want to know about the results of research using their data.*

The European Patients' Forum also asks for more clarity and harmonisation on data ownership at European level.

Patient organisations have often referred to patients 'owning' their data. This was not always intended in a legal sense; the legal implications of terminology are still being discussed (for example in relation to GDPR). The intention is to ensure that patients are considered owners of their data in a moral sense, regardless of the legal framework. They should have a right to participate in decisions about what happens with their data, including governance and policy making.

Chapter 5. Use of real-world data and evidence: key points

1. Collecting ‘real-world data’ – information collected from routine use of medicines in the community – is essential for making sure that medicines continue to be used to their best effect.

2. Strong collaboration between patient communities, regulators, data providers, and the pharmaceutical industry leads to better collection of real-world data – meaning data on the effectiveness and safety of medicines.

3. Patients should be seen as partners in deciding what information is collected, how it is collected, and how it is used. Care is needed to involve diverse patient views.

4. Patient-engagement frameworks for real-world data have been developed - but there is scope to improve them and for implementing them more fully.

5. Patients’ involvement in generating real-world data – often using emerging technologies – should continue to be expanded.

CIOMS WG XI Consensus Report Part II

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ISoP Advisory Board
CIOMS-CoRE Webinar
31st January 2023

Objectives
Patient involvement in

- Product labelling, which includes information given to patients with medicines.
- The development and communication of urgent patient safety information.
- The design, development and implementation of RMMs.
- Patient and public involvement in developing clinical practice guidelines
- Challenges of patients living in remote or deprived communities.
- The impact of COVID-19 pandemic and the voice of the patient.

Chapter 6. Product Labelling

Disclaimer and declaration of conflict of interest

Manal Younus

BPharm, MSc, PhD.

Declaration of Conflicts of Interest

- Full-time employee of Iraqi MOH
- I have no conflicts of interest to declare

Disclaimer: The views expressed in this presentation reflect the personal views of the author and do not necessarily reflect the views of the authors' employers Iraqi MOH nor ISoP, nor any other institutions the author may otherwise be collaborating with.

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6.6 Principles for Patient Engagement in the Development of Patient Labelling

Principle 1	Principle 2	Principle 3
Involve patients in the design of the patient label	Include patients in the iterative testing of patient labelling materials	Engage patients to evaluate the effectiveness of patient labelling after authorization

Chapter 7. Rapid Safety Communication

Chapter 7. Rapid Safety Communication: Key Points

Patients can contribute to urgent safety communication in different ways:

1. Taking part in decisions about which new safety issues patients need to be quickly alerted about.
2. Providing the patient's perspective on what needs to be communicated and how it can be communicated.
3. Using the different communication channels available to patient organisations to send out urgent safety communication.
4. Responding to questions or moderating discussions among patient organisation members about the urgent safety information.
5. Providing input from an early stage through pre-set processes.
6. Providing input on the appropriate information and terminology (lay language) in the information to be sent out
7. Providing input into the development of a glossary of terms specific to a disease and set of treatments translated into plain language and the language to be used for potential future communications.

Chapter 8. Additional Risk Minimization

8.1		**Risk minimization**
	8.1.1	How risk is minimized
8.2		**Patient involvement in additional risk minimization**
	8.2.1	When to involve patients in additional risk minimization
	8.2.2	Ways of involving patients in additional risk minimization measures
8.3		**How to involve patients at each step of the additional risk minimization process**
	8.3.1	Decision to introduce additional risk management measures
	8.3.2	Designing additional risk management measures
	8.3.3	Developing additional risk management measures
	8.3.4	User testing additional risk minimization measures
	8.3.5	Implementing additional risk minimization measures
	8.3.6	Evaluating additional risk minimization measures
8.4		**How regulators involve patients in additional risk management measures**
	8.4.1	European Union
	8.4.2	USA
	8.4.3	Japan
8.5		**Conclusions and recommendations**

Annex 1	**Additional details of the risk minimization process in the EU and US**
Annex 2	**Detailed information on routine and additional risk minimization** • Description of routine risk minimization measures • Description of additional risk minimization measures (aRMM) • Controlled medicine distribution and additional risk management measures • Types of controlled medicine distribution and additional risk management measures
Annex 3	**Example of interview questions to collect patient views on additional risk minimization**
Annex 4	**Failure modes and effects analysis for risk minimization**

Chapter 8. Table 10. Questions Based on the General Patient Treatment Pathway to Obtain Patient Perspectives

Patient and treatment selection	Dispensing of product	Product use	Follow-up

• What does a patient need to know about how a patient is selected for treatment?
• How does the prescriber select suitable treatment for the patient?
• What do patients need to know about testing (e.g. screening or biomarker) to identify those more vulnerable to a risk?
• What do patients need to know about vaccinations before and during treatment?
• Do healthcare providers other than the prescriber interact with the patient?
	• Does the patient or caregiver need pre-treatment instructions?
	• Should the patient be counselled about:
	  • Nature of the risks?
	  • Signs and symptoms of the risks?
	  • How to take the product?
	• Will patients or caregivers receive the medicine on time?
	• What do patients think about the product being dispensed in a specific healthcare setting (e.g. inpatient or infusion centre)?
		• How is the product administered?
		• What is the treatment setting?
		• Can a patient self-administer the product (e.g. when medicine needs to be reconstituted or Injected)?
		• Does the amount of medicine needing to be taken change over time (e.g. weight based dosing)?
		• How difficult is it to follow the instructions for using the product?
		• Will patients understand and follow product use instructions?
		• Should patients be observed or monitored during administration
			• Are patients aware of the risks?
			• Are patients aware of signs and symptoms of risks?
			• Would early recognition of signs and symptoms enable the patient to act to reduce severity of the risk?
			• Can the patient act to prevent the risk?
			• Will the patient attend monitoring appointments, follow-up visits?
			• Will the patient adhere to laboratory testing and monitoring requirements?

Source: CIOMS Working Group WG XI

Chapter 8. Additional Risk Minimization: Key Points

1. Every authorised medicine has potential benefits and potential risks; its benefits must outweigh its risks for it to be licensed and remain on the market.
2. Some medicines have risks which need more than the usual risk minimisation measures.
3. Additional risk minimisation measures may place an additional burden on patients and on the healthcare system. This means that the measures need to be proportionate to the relevant risk.
4. Additional risk minimisation measures should be designed to fit easily into the healthcare system.
5. Patients can provide invaluable insights into the best way to minimise risks. This means they should be involved at all stages when considering additional risk minimisation measures.

Chapter 9. Clinical Practice Guidelines

Chapter 9. Figure 10. Patient and Public Involvement During Guideline Development

Source: CIOMS Working Group WG XI

Chapter 9. Clinical Practice Guidelines: Key Points

1. Patient and public involvement is important for creating a clinical practice guideline of high quality.

2. An effective process for involvement ensures that patients and members of the public are able to share their views – and that the guideline takes account of these views.

3. The principal steps of involving patients and members of the public in the guideline development process are:
   - Informing them about the guideline for making health decisions;
   - gathering the views of a broad group of patients or members of the public; and
   - inviting patients and members of the public to join the group that creates the guideline.

4. There are several ways to achieve effective patient and public involvement. The choice of the path depends on the guideline developer's goals and resources.

5. Effective processes to recruit and support patients or the public are vital to make sure that patients can contribute their views freely. The recruitment process should be transparent, and selection should follow pre-set criteria.

Chapter 10. Low- and Middle-Income Countries

Chapter 10. Low- and Middle-Income Countries: Key Points

1. The principles for involving patients in low- and middle-income countries should be no different from that in high-economy countries.

2. There are specific challenges in low- and middle-income countries – making it difficult to fully involve patients in the development, regulation and safe use of medicines.

3. Civil society, people working in medicine research and development, government, international institutions, and non-governmental organizations can all support patient involvement in low- and middle-income countries.

4. The following actions can improve patient involvement in low- and middle-income countries:
    a. Improved health literacy of the general public and respect from healthcare providers for patients as equal partners in the fight against disease;
    b. Communicating openly and in plain language that encourages two-way discussion;
    c. Developing laws and policies that fully involve the participation of patients in healthcare decisions that affect them and their communities;
    d. Sharing knowledge and success stories between patient organisations locally and internationally;
    e. Enforcing highest ethical standards for medicines research that fully respect patients' needs;
    f. Building capacity by engaging with international patient organisations – as well as learning from experience in high-economy countries.

Chapter 11. Pandemic Considerations

Chapter 11. Pandemic Considerations: Key Points

1. Previous pandemics and the current severe acute respiratory syndrome coronavirus-2 (SARS-CoV 2) pandemic have highlighted the need for patient involvement in their management.

2. There is much experience of patient involvement in the human immunodeficiency virus (HIV), which emerged in the 1980s. Here, the patient voice had a great impact on therapeutic interventions and clinical trials.

3. Public health measures to stop the spread of SARS-CoV-2 have been challenging because of how people behave and because of miscommunication.

4. Several factors have led to vaccine hesitancy and antivaccination attitudes. This makes it likely that the virus will continue to circulate.

5. There will likely be another pandemic, possibly an entirely new infection. We must make use of what we have learned so far to develop more effective ways of communicating about pandemics across the world.

Appendices

1		**Glossary**
2		**Case studies**
	A	Medication formulation created to meet patients' and doctors' needs (AdrenalNET)
	B	A regulatory agency involving patients; public hearing on valproate (EMA)
	C	Pilot collaboration between Lareb and a patient organisation in communicating a signal (Lareb)
	D	Creating partnerships between industry and patient groups for therapy development (Roche)
	E	Example of a pharmaceutical company working with patients to develop an additional risk minimisation measure
	F	Engaging patients in early development plans for a novel treatment (Takeda)
	G	Patient activism to counter AIDS denialism and improve access to HIV medicines in South Africa
3		**CIOMS Working Group XI statement**
4		**CIOMS Working Group XI membership and meetings**
5		**List of commentators**

Each case study describes:

Purpose and objective of the case study

Pharmacology

Indication/disease treated

Stage of the drug development lifecycle

Why were patients involved?

How was contact established with the patients?

What did the patients do?

Was the process adjusted to the patients' needs?

If patients were asked to help disseminate information, how was it done?

Did the patients receive payment or compensation?

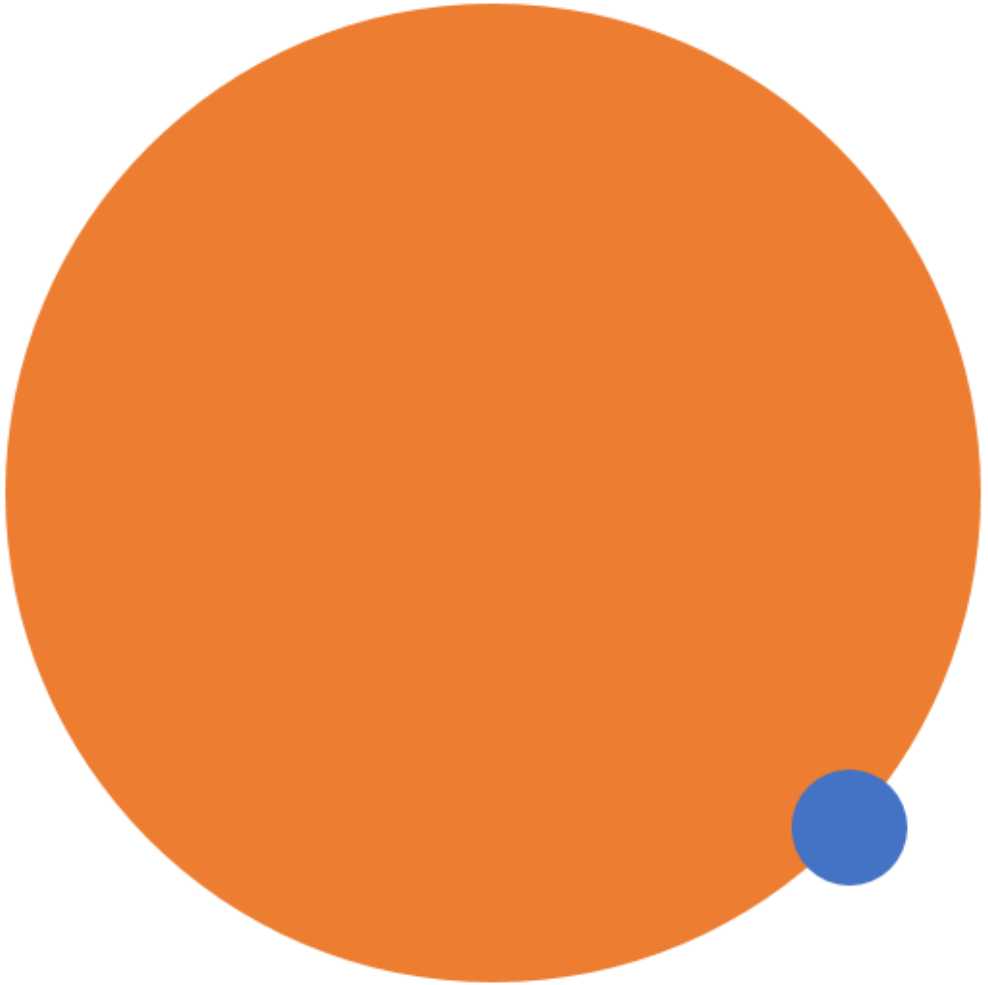
Were any patient requests or recommendations discarded and why?

Conclusion

Contact details

Thank you!

- *Patient involvement in the development, regulation and safe use of medicines* describes and promotes the idea that patients should be involved throughout the medicine's life – from their development, through regulation to ongoing safe use in everyday healthcare. It describes where we are, and a path to where we need to go.
- This isn't a Communications Department exercise. It is an enterprise-wide responsibility



The patient voice is not a passive voice

"A pessimist sees the difficulty in every opportunity; an optimist sees the opportunity in every difficulty."

Winston Churchill

“Change is not required. Survival is not mandatory.”

-W. Edwards Deming

Thank you!