Introduction

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

Pharmacovigilance

- 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
<table>
<thead>
<tr>
<th>Working Group topic and starting date</th>
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<tr>
<td>1. MedDRA Labelling Groupings. <em>Started April 2019</em></td>
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<td>2. Benefit-Risk Balance for Medicinal Products. CIOMS WG XII. <em>Started September 2019</em></td>
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<td>3. Real-World Data and Real-World Evidence in Regulatory Decision-Making. CIOMS WG XIII. <em>Started March 2020</em></td>
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<td>4. Severe Cutaneous Adverse Reactions (SCARs). <em>Started February 2021</em></td>
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<td>5. Recommended Standards of Education and Training for Health Professionals Participating in Medicines Development. <em>Started April 2021</em></td>
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<td>7. Artificial Intelligence in Pharmacovigilance. CIOMS WG XIV. <em>Started May 2022</em></td>
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This webinar is about introducing the new CIOMS report

Patient involvement in the development, regulation and safe use of medicines

Report of the CIOMS Working Group XI

Council for International Organizations of Medical Sciences (CIOMS)

Geneva 2022
How we do it?

Two speakers

Elisabeth Oehrlein

Panel discussion moderated by

Manal Younus

Peter Pitts
Thank you!

And now over to our speakers and moderator to continue
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

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Chapter 9: Clinical practice guideline

Chapter 10: Low- and middle-income countries

Chapter 11: Pandemic considerations

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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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<th>Name</th>
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<tr>
<td>Schaefer, Corinna</td>
<td>World Medical Association (WMA)</td>
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<td>Edwards, Brian*</td>
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<td>Olsson, Sten</td>
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<td>Lindquist, Marie</td>
<td>Uppsala Monitoring Centre (UMC)</td>
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<td>Bateman-House, Alison</td>
<td>Grossman School of Medicine, NYU Langone Health, US</td>
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<td>Raynor, Theo</td>
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<td>Harmark, Linda</td>
<td>The Netherlands Pharmacovigilance Centre Lareb</td>
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<tr>
<td>Star, Kristina*</td>
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<td>Younus, Manal*</td>
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### Patient representatives

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<tr>
<th>Name</th>
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<tr>
<td>Boutin, Marc**</td>
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<tr>
<td>Dedes, Nikos</td>
<td>European AIDS Treatment Group (EATG)</td>
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<td>Houyéz, François</td>
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<td>Immonen, Kaisa</td>
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<td>Kamoga, Regina</td>
<td>Community Health and Information Network (CHAIN), Uganda</td>
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<td>Leeson-Bevers, Kerry</td>
<td>Alström Syndrome UK</td>
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<td>Selimi, Kavaldip</td>
<td>International Alliance of Patients’ Organizations (IAPC)</td>
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### Regulators

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<td>Arsenault, Denis*</td>
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<td>Bere, Nathalie</td>
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<td>de Boer, Antonius</td>
<td>Medicines Evaluation Board (MEB), The Netherlands</td>
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<td>Foy, Mick*</td>
<td>Medicines and Healthcare products Regulatory Agency (MHRA), UK</td>
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<td>Oi, Tsunehiro*</td>
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<td>U.S. FDA</td>
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Acknowledgements

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Asiimwe, Alex .................................. Bayer
Bhayat, Fatima* ................................ Takeda
Boedding, Matthias ............................ Merck
Blackburn, Stella ............................... IQVIA
Clary, Cathryn* ................................ Novartis
Da Silva, Barbara** ............................ AbbVie
Garrigan, Charles .............................. Janssen
Heaton, Stephen ............................... Independent consultant, formerly Bayer

Harrison, Beverly ............................ Janssen
Kaehler, Stefan ................................ Celgene, Bristol Myers Squibb
Kugener, Veronique** ....................... Takeda
MacCracken, Christine* ..................... Janssen
Metcalf, Marilyn .............................. GLaxoSmithKline
Noel, Rebecca ................................ Eli Lilly
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Vaidya, Pujita .................................. Amgen
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Stürchler, Christine .......................... Novartis
Webster, Peggy** ............................. Takeda
Wilkins, Jamie* ................................ Pfizer

*Acknowledged for their personal contributions
**Acknowledged for their professional contributions
Chapter 1: Introduction
Chapter 2: Landscape
Chapter 3: Guiding principles
Chapter 4: Advancing treatments
Chapter 5: Use of real-world data and evidence
• **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.

• **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.

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
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
Chapter 2: Landscape

2.1 Opportunities for patients to engage
   2.1.1 Patient organizations
   2.1.2 HIV/AIDS activism
   2.1.3 Rare disease patient advocacy

2.2 Patient-centricity in medicine development
   2.2.1 Patient–centered outcomes
   2.2.2 Patient-focused medicine development
   2.2.3 Barriers to meaningful engagement
   2.2.4 Overcoming regulatory and legal uncertainties
   2.2.5 Promoting culture shift
   2.2.6 Open communication and information
   2.2.7 Patient engagement in advancing medicine safety

2.3 Continuing culture shift

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

Culture
- Using a science-based approach
- Financial risks
- Organizational culture

Regulatory/Legal Uncertainty
- What and how information is reviewed by FDA
- Patient interactions

Fundamental Barriers to Meaningful Patient Engagement

Communication
- Proprietary information
- Translation and patient acknowledgement
- Visibility

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:
   - Identifying patient-centered outcomes (also called patient-relevant outcomes);
   - Participating in regulatory review;
   - Contributing to constructing, reviewing and disseminating medicines information;
   - 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
- Discarding 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

3.4. Training of stakeholders for patient engagement activities

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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
- 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
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

## 4.1 Purpose of patient engagement in treatment selection

## 4.2 Patient engagement and unmet needs
- **4.2.1** Reaching out to patients
- **4.2.2** Special considerations

## 4.3 Patient engagement in preclinical or early clinical development

## 4.4 Patient engagement in clinical development
- **4.4.1** Individual choices

## 4.5 Challenges in clinical development
- **4.5.1** Communicating clearly
- **4.5.2** Including diverse and underserved patients
- **4.5.3** Balancing digital technology and inclusiveness
- **4.5.4** Patient engagement takes time
- **4.5.5** Finding and engaging harder-to-reach patients
- **4.5.6** Overburdening patient organizations
- **4.5.7** Providing clinical trial information to patients
- **4.5.8** Engaging patients who cannot provide direct input
- **4.5.9** Compensating patients for their engagement

## 4.6 How to engage

## 4.7 Patient engagement in patient preference studies

## 4.8 Patient engagement in regulatory review
- **4.8.1** Purpose of involving patients in regulatory processes
- **4.8.2** Patient involvement at key milestones during medicine regulation
- **4.8.3** Contributions on disease and product specific questions
- **4.8.4** Ad hoc advisory committees and panels
- **4.8.5** Communication
- **4.8.6** Ongoing patient engagement forums
- **4.8.7** Training capacity building
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

<table>
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<tr>
<th>Stage: Ongoing</th>
<th>Unmet need</th>
<th>Early development</th>
<th>Clinical development</th>
<th>Regulatory review</th>
<th>Healthcare delivery (Safety monitoring)</th>
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<td><strong>Patient</strong></td>
<td>Company needs</td>
<td>Establish research priorities</td>
<td>Develop patient-relevant outcomes</td>
<td>Contribute to dossiers/reviews</td>
<td>Learn about treatments</td>
<td>Create/review non-promotional information</td>
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<td>Product needs</td>
<td>Describe living with disease</td>
<td>Contribute to protocol design</td>
<td>Contribute to benefit-risk profile</td>
<td>Contact developers about promising products for compassionate use</td>
<td>Create/review non-promotional information</td>
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<td></td>
<td>Develop products</td>
<td>Establish standard of care</td>
<td>Contribute to regulatory development process</td>
<td>ASTEX (Enhancing Small-molecule Drug Discovery for Regulatory innovation and excellence)</td>
<td>Talk about treatments and goals with HCP</td>
<td>Create/review non-promotional information</td>
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<td></td>
<td>Provide information to patients</td>
<td>Describe needs, goals, and wants</td>
<td>Co-create/review research plans</td>
<td>Co-create/review information for patients</td>
<td>Tell HCP/sponsor/regulator about side effects</td>
<td>Create/review non-promotional information</td>
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<td>EMA/NIH/NIH Reuse</td>
<td>FDA/NIH/mySquares</td>
<td>Engage conversations with developers following a safety signal once the product is on the market. This may be the first day between patients and drug developers.</td>
<td>Create/review non-promotional information</td>
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<td>Create/review non-promotional information per guidance</td>
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<td><strong>Healthcare professionals</strong></td>
<td>Establish clinical guidelines</td>
<td>Talk with/patient about needs, goals, and wants</td>
<td>Inform patients about clinical trials</td>
<td>Give input on current treatment</td>
<td>Learn about safety and appropriate use of product</td>
<td>Co-create/review/distribute non-promotional materials</td>
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<td></td>
<td>Develop natural history studies</td>
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<td>and eligibility for clinical trials</td>
<td>for patients</td>
<td>Report side effects promptly</td>
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<td><strong>Developers</strong></td>
<td>Joint research priority partnership, e.g., The James Lind Alliance (see section 5.3.7)</td>
<td>Discus with patients their needs, goals, and wants</td>
<td>Support patients throughout the trial and give regular feedback</td>
<td>Talk about standard treatment</td>
<td>Engage with patients to establish treatment guidelines</td>
<td>Co-create/review/distribute non-promotional materials</td>
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<td>Co-create with patients or request patient review of research plans</td>
<td>Incorporate needed changes</td>
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<td>Incorporate needed changes</td>
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<td><strong>Regulators</strong></td>
<td>Early dialogue/hearing with patient and patient groups, e.g., EMA's Innovation Task Force and FDA's Patient Listening Sessions</td>
<td>Involve/patient advocacy groups in development plans</td>
<td>Co-create with patients and provide guidance on including patients' input in treatment development</td>
<td>Include patient feedback to patients</td>
<td>Monitor safety and effectiveness of treatments in patient-friendly ways</td>
<td>Co-create/review/distribute non-promotional information</td>
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<td>Involvement in orphan designations</td>
<td>Involve patients in development plans</td>
<td>Co-create with patients and provide guidance on including patients' input in treatment development</td>
<td>Monitor safety and effectiveness of treatments in patient-friendly ways</td>
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<td>Involvement in scientific advice</td>
<td>Collaborate with patients</td>
<td>Co-create with patients and provide guidance on including patients' input in treatment development</td>
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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.\textsuperscript{18,26}

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.\textsuperscript{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, EUPATH-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’.\textsuperscript{28}
- PREFER delivered expert and evidence-based guidance on when and how to design and conduct a patient preference study.\textsuperscript{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.\textsuperscript{29}
Example method to gather information about patient experiences and unmet needs

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);

IMIPREFER, 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.
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

5.1 Patient involvement in generating real-world data on medicines
  5.1.1 Patient and regulators
  5.1.2 Patient and industry
  5.1.3 Patients and healthcare professionals
  5.1.4 Patients and patient organizations

5.2 Patient data and their use in post-marketing environment
  5.2.1 Collecting patient data

5.3 Challenges and opportunities for patient engagement in the development and use of real-world data
  5.3.1 Informed consent
  5.3.2 Patient privacy
  5.3.3 Data ownership or control
  5.3.4 Patient engagement
  5.3.5 Patient voice in regulatory advances
  5.3.6 Patient engagement with healthcare providers
  5.3.7 Patient and researchers
  5.3.8 Vulnerable populations
  5.3.9 Social media
  5.3.10 Health literacy and user-friendly interfaces

5.4 Conclusion
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

Dr Manal Younus
Head of Iraqi Pharmacovigilance Centre, MOH
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.
Chapter 6. Product Labelling

6.1 Summary

6.2 Introduction

6.3 Sources of medicinal product risk and safe use of information for patients
   6.3.1 Product labeling
   6.3.2 Additional risk minimization materials
   6.3.3 Promotional materials from pharmaceuticals companies
   6.3.4 Other sources of patient-targeted medicinal product benefit-risk information

6.4 Initiatives to improve the quality of patient labelling

6.5 High-quality patient-centered patient labelling

6.6 Principles for patient engagement in the development of patient labelling

6.7 Evaluating the effectiveness of patient labelling

6.8 Future directions for patient labelling

Annex 1 Product labelling for patients – requirements worldwide

Annex 2 Comparison of content requirements

Annex 3 Initiatives to improve patient labelling

Annex 4 Best practice recommendations for patient labelling information
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**
Involve patients in the design of the patient label

**Principle 2**
Include patients in the iterative testing of patient labelling materials

**Principle 3**
Engage patients to evaluate the effectiveness of patient labelling after authorization
## Chapter 7. Rapid Safety Communication

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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

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### 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

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

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

9.1 Introduction

9.2 Guidelines

9.3 A quality criterion for clinical practice guidelines

9.4 Core principles

9.5 Rationale and methods

9.6 Involvement strategies
  9.6.1 Consultation strategies
  9.6.2 Participation
  9.6.3 Communication

9.7 Representativeness

9.8 Patient and public involvement in guideline development

9.9 Patient and public involvement in effective recruitment
  9.9.1 Nomination
  9.9.2 Open recruitment

9.10 Training and support

9.11 Documenting and managing conflict of interest

9.12 Barriers to patient and public involvement

9.13 International patient and public involvement activities

9.14 Effect of patient and public involvement

9.15 Key components of successful patient and public involvement
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.

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.
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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.

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

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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.
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!