Stakeholder engagement at EMA

EURORDIS Open Academy

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Public and Stakeholder Engagement Department

What does a medicines regulatory agency do?

What we do

Protect human and animal health

- Facilitate development and access to medicines
- Evaluate applications for marketing authorisation
- Monitor the safety of medicines across their life cycle
- Provide reliable information on human and veterinary medicines to patients and healthcare professionals

Who we are

7 Scientific Committees

1 Management Board

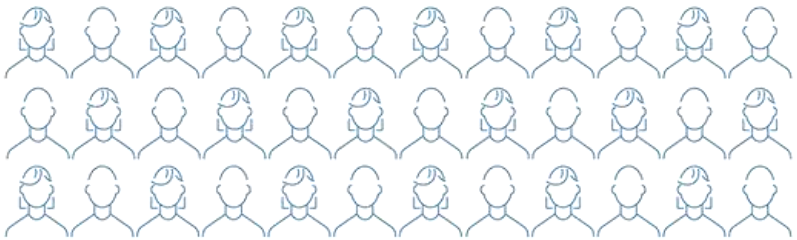
CHMP
CVMP
COMP
HMPC
PDCO
CAT
PRAC

27 Member States' representatives

4 Civil society representatives

2 European Commission representatives

2 European Parliament representatives

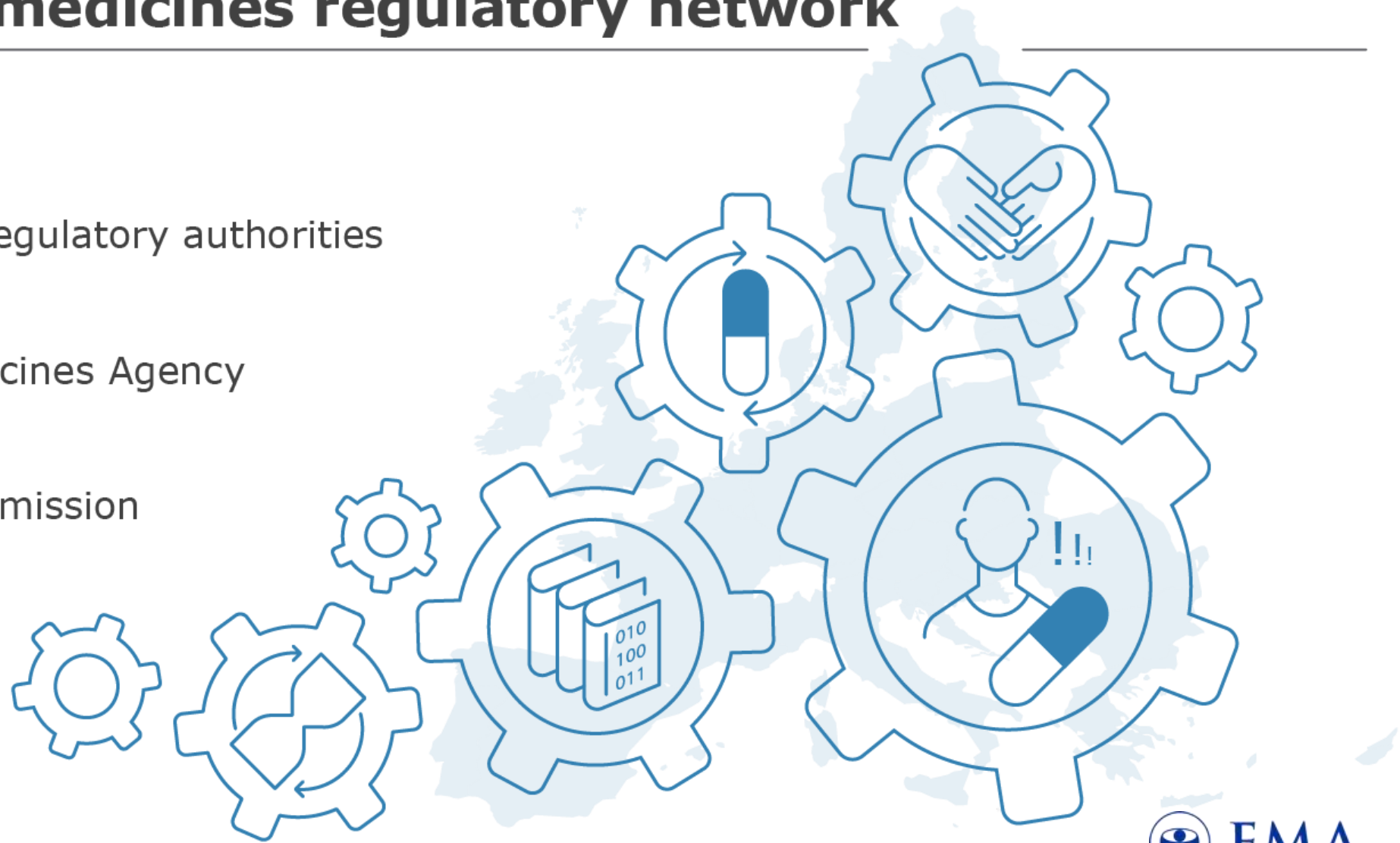


~800 staff members

EMA

The European medicines regulatory network

- ~50 national regulatory authorities
- European Medicines Agency
- European Commission



Why would EMA want to engage with stakeholders?

Transparency and added value

- Description of patient/HCP input into EMA activities
- Proposals for improvements included in next work-plan
- Annual report to EMA Management Board

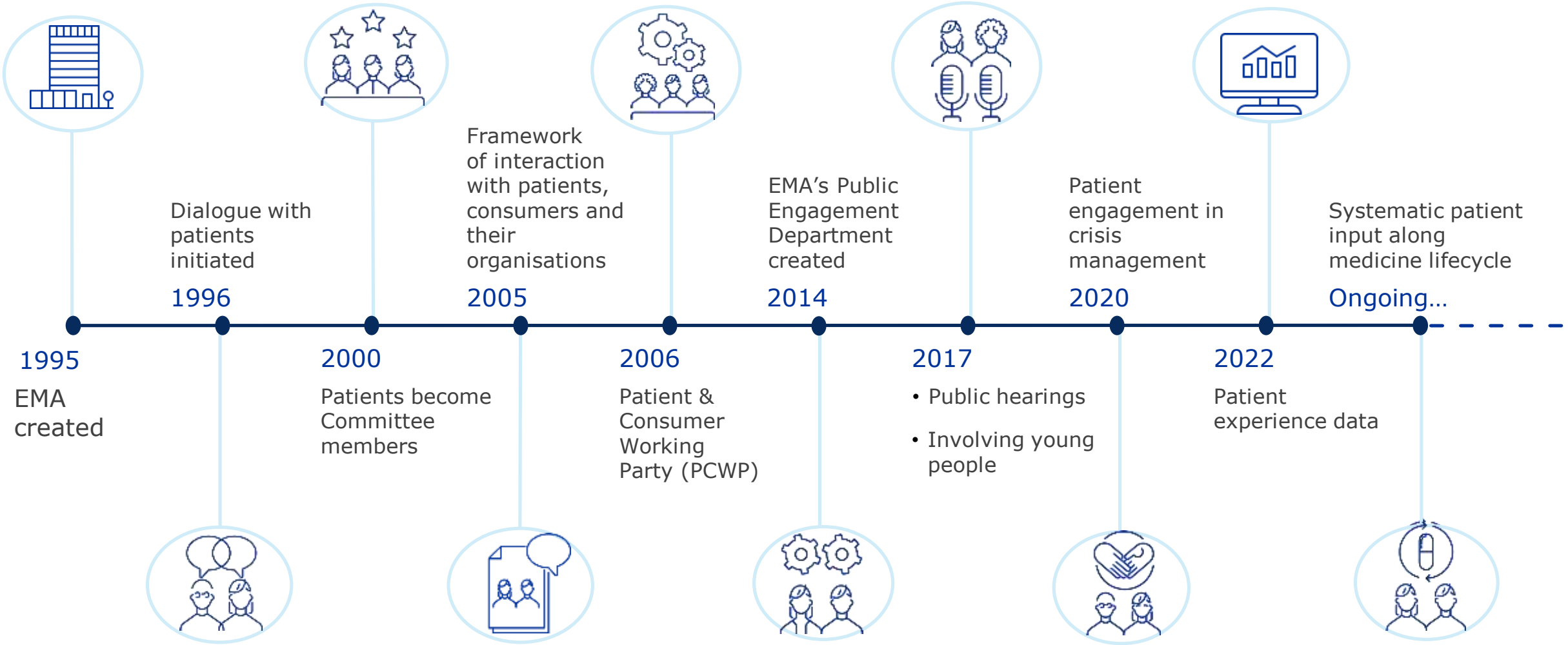
Annual reports with summaries of feedback

Review of documents

- ❖ Comments and suggestions by patients incorporated into published documents
- ❖ Template structure changed

How does EMA engage with stakeholders?

Interaction with patients and consumers:



a progressive journey…

EMA activities with patients: Engagement Framework

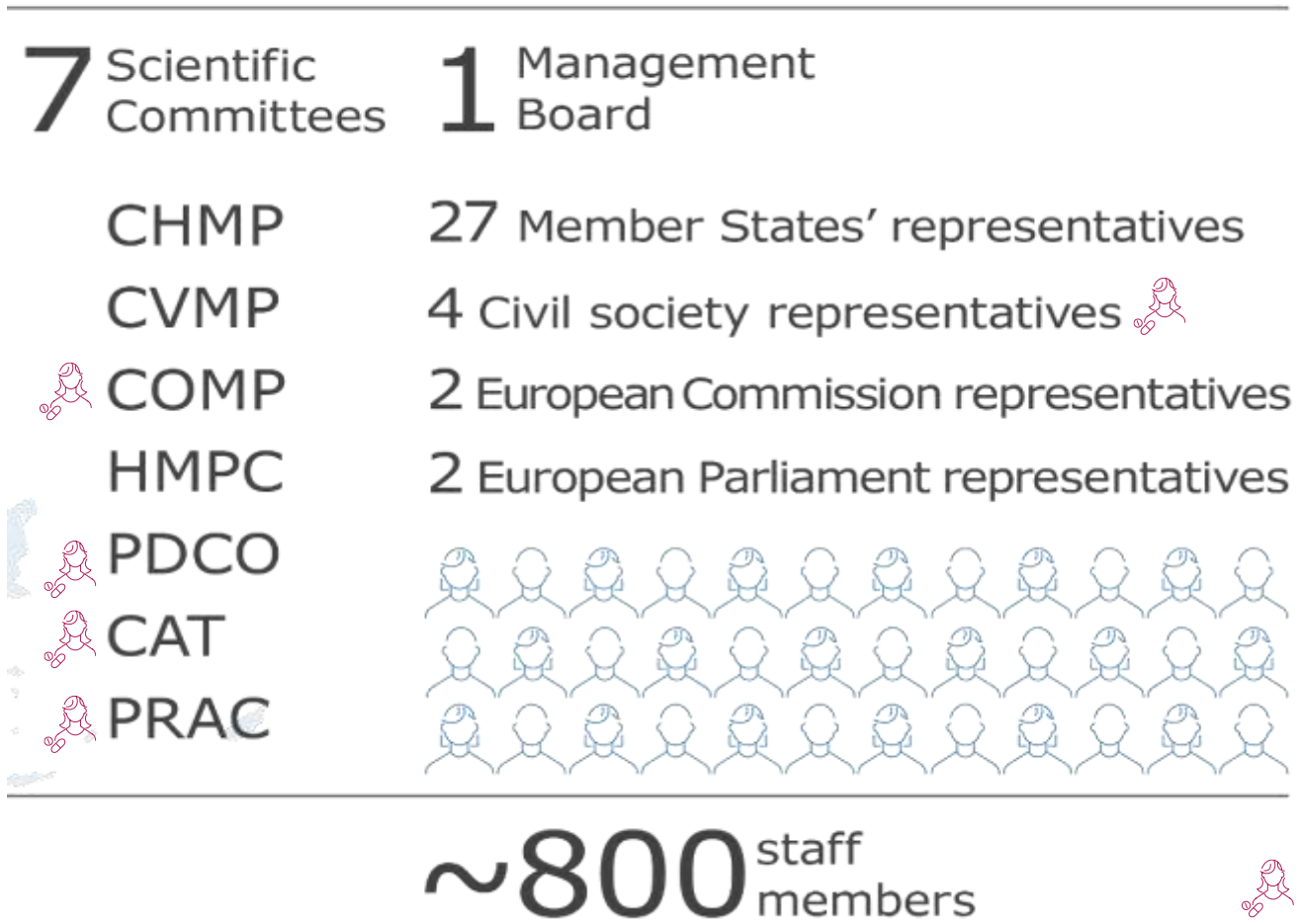
1. A **network** of European patients' and consumers' **organisations**;
2. Patients' and Consumers' Working Party (**PCWP**);
3. A pool of **individual** patients, consumers or carers,
4. **Capacity-building** and training
5. Range of **engagement methodologies** enabling patients and consumers to be included along the medicine's regulatory lifecycle
6. Development of guidance on the generation, collection and use of **patient experience data**;
7. Interaction with the **EU Regulatory Network**.

Categories of representation

Representing their *community*	• Management Board • EMA Scientific Committee Members
Representing their *organisations*	• Working Party (PCWP and HCPWP) • EMA consultations (policies and guidelines) • Workshops
Representing themselves as *individuals*	• Scientific Advice / Protocol Assistance Procedures • Scientific Advisory/ad hoc expert Groups • Scientific Committee consultations • Review of documents

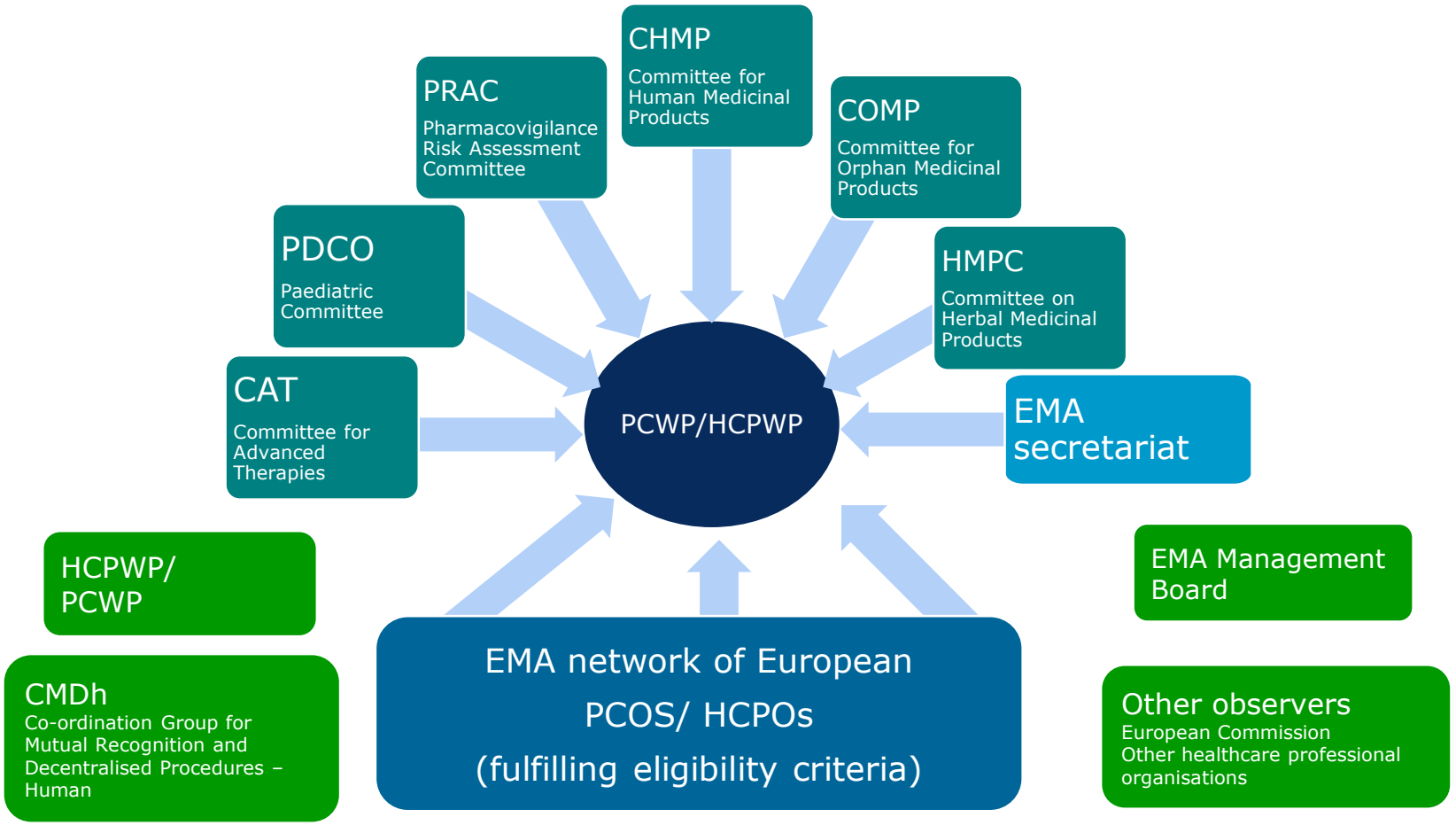
Patients and healthcare professionals are engaged in medicine-related and non-medicine related activities.

EMA scientific committees and Management Board



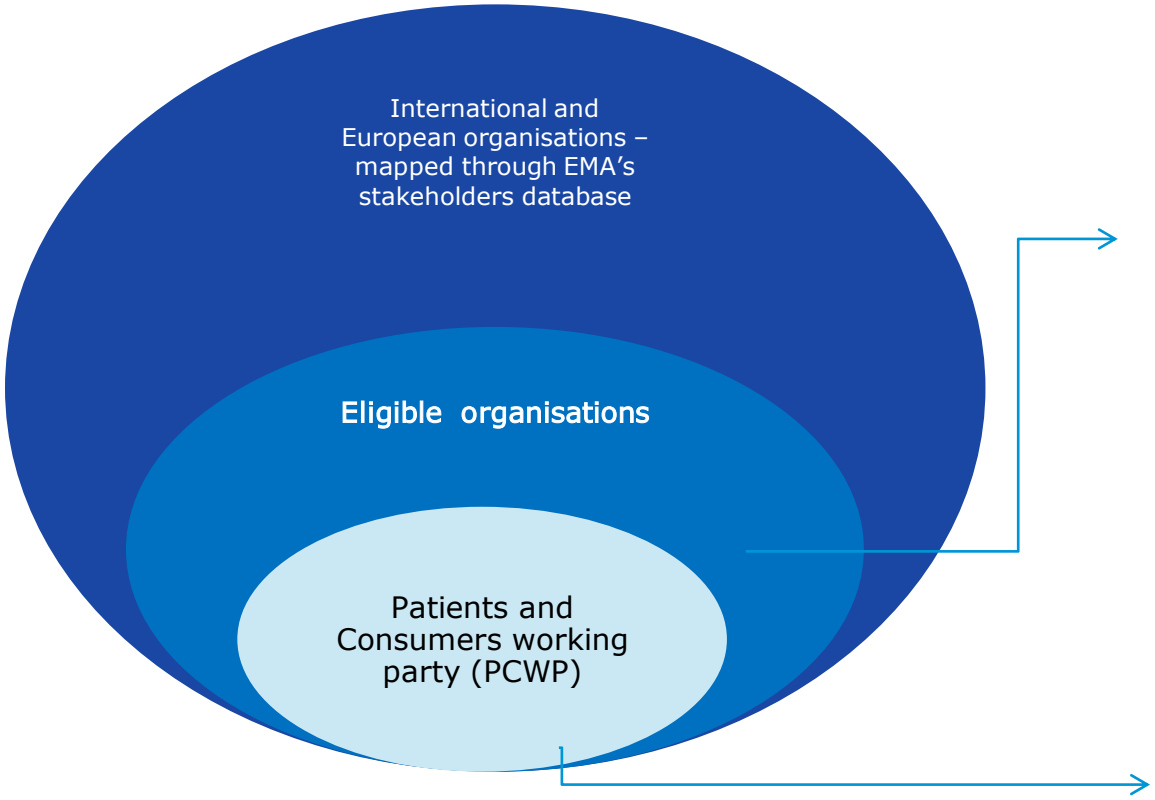
Representing their community

PCWP and HCPWP

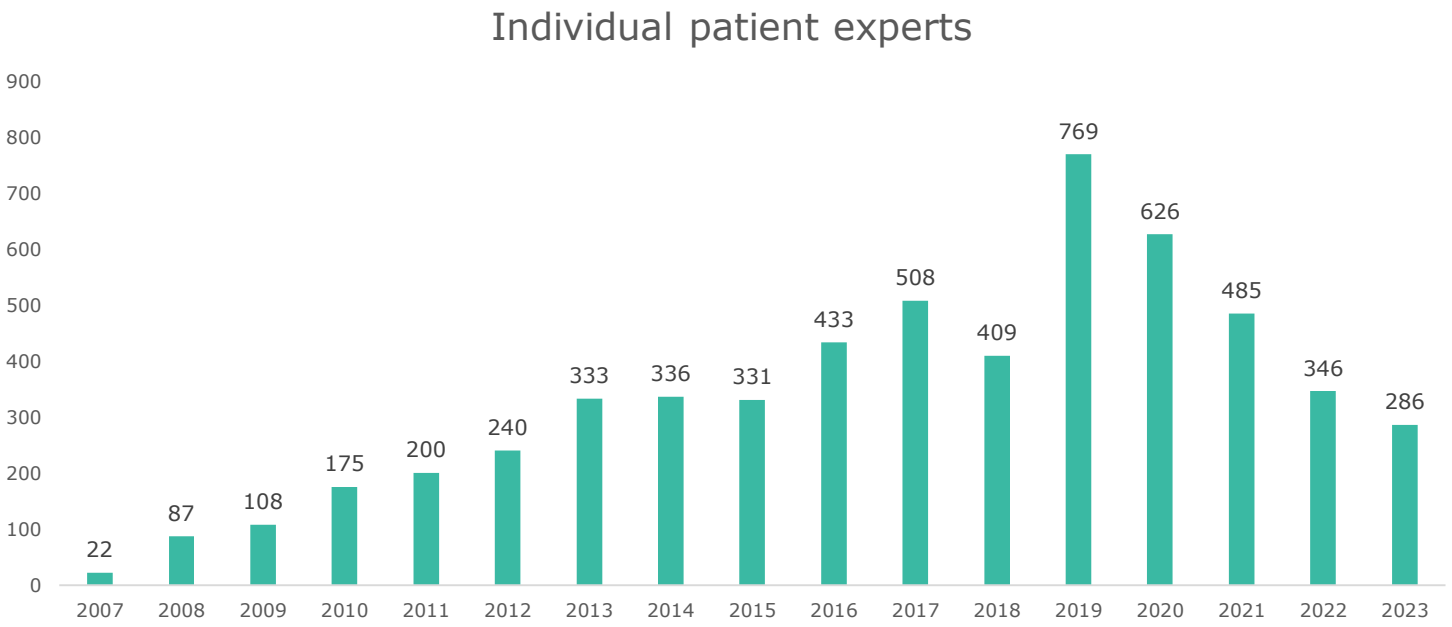


- Balanced representation of different types of PCOS/HCPOs
- 25 members of the WP are selected from the list of eligible organisations
- Each human scientific committee nominates a member
- Up to 3 meetings/year

Sources for reaching out to patients



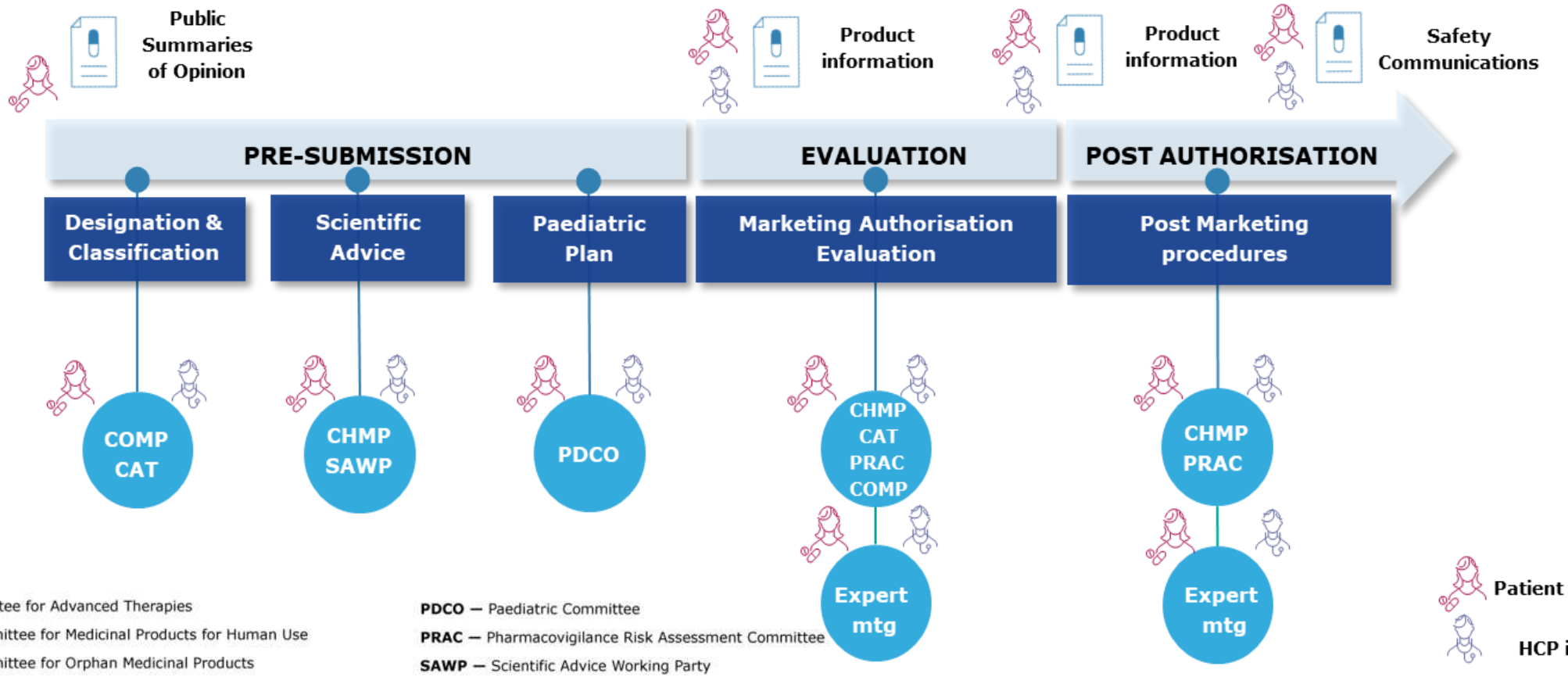
Patients as individual experts in medicine-specific activities



Scientific Advice / Protocol Assistance Procedures
Scientific Advisory/ad hoc expert Groups
Scientific Committee consultations
Review of documents

Bringing expertise into the EU medicines regulatory system

Involvement along the medicine lifecycle at EMA



CAT — Committee for Advanced Therapies
CHMP — Committee for Medicinal Products for Human Use
COMP — Committee for Orphan Medicinal Products

PDCO — Paediatric Committee
PRAC — Pharmacovigilance Risk Assessment Committee
SAWP — Scientific Advice Working Party

Engagement and support

One size does not fit all!

Methodologies for engagement

Face to face meeting

oral explanations - scientific advice - SAG

In writing

written responses to scientific advice - surveys

Training and support

- EMA training day
- Information sheets
- Videos on EMA website
- Information on webpages
- One to one support

Challenges for patient involvement

- Finding suitable patients (e.g. language barrier, availability)
- Ensuring comprehensive, tailored training to facilitate and enhance participation
- Provide a clear definition of patients' role in the different activities to manage expectations
- Competing interests
- Representativeness

Criteria and transparency

Organisation representatives	Individual Experts
EMA 'eligibility' criteria	**Declaration / assessment of Interests**
Transparent on the funding of the organisation ▶ Legitimacy ▶ Mission/activities ▶ Representation ▶ Structure ▶ Accountability ▶ Transparency	**Confidentiality undertaking** Identification through European network of registered organisations and EMA database of individuals

Organisations can become EMA eligible organisations by fulfilling certain criteria.

Individual experts must complete a declaration of interest and confidentiality undertaking

Why would patients want to engage with EMA?

How can you be involved?

Committee member Management Board

- European Commission publishes Expressions of interest on their website and nominates civil society committee members

Eligible organisation Member of PC/HCP-WP

- Register your organisation with EMA – criteria on website
- PC/HCP-WP membership – 3 year mandate based on representation.

Individual patient expert with EMA

- Register via link
- Email anyone of the team at public-engagement@ema.europa.eu
- Remuneration of experts

What difference does stakeholder engagement make?

Patient engagement – added value and impact

- Scientific Advice

❖ 4-year study published

❖ Added value of patient input quantified and demonstrated

CHMP early contact

❖ Early consultation on issues relevant to patients/carers

❖ Integrated input into assessment report

Review of documents

❖ Comments and suggestions by patients incorporated into published documents

❖ Template structure changed

Safety monitoring

❖ Public hearings – recommendations leading to risk minimisation measures

EMA

- Any questions?

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European Medicines Agency

Send a question via our website www.ema.europa.eu/contact

EMA

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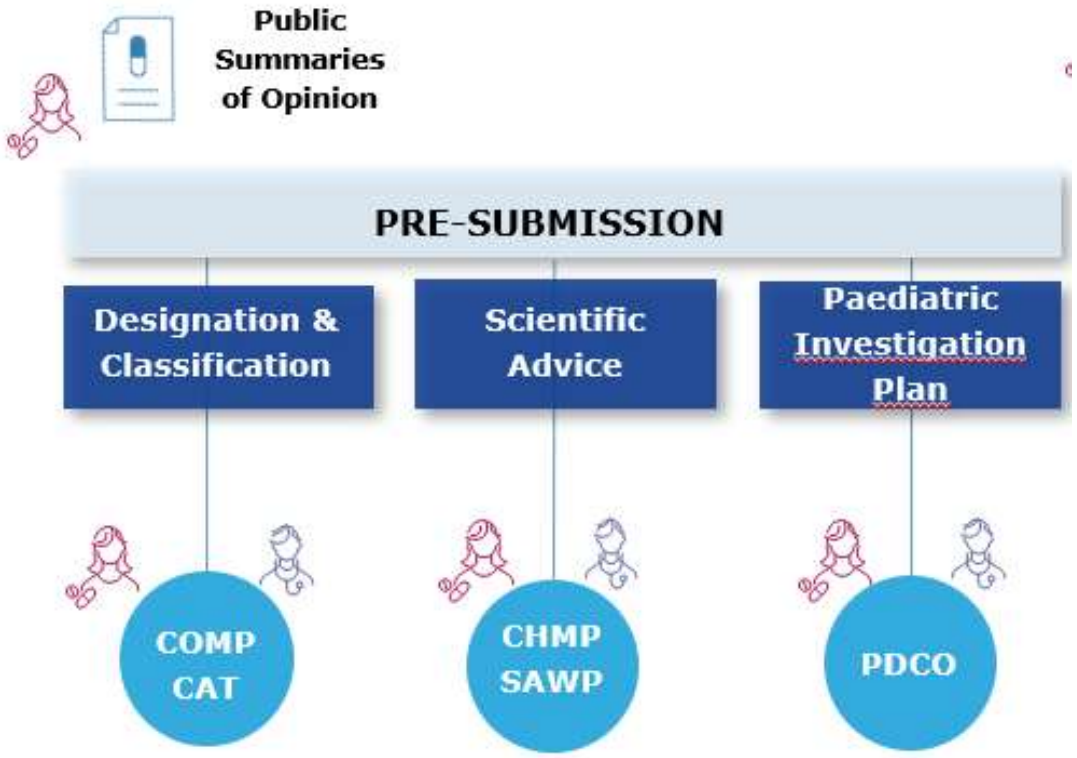
EUROPEAN MEDICINES AGENCY

Evaluation and post-authorisation activities involving patients

Maria Mavris, Patient Liaison, EMA

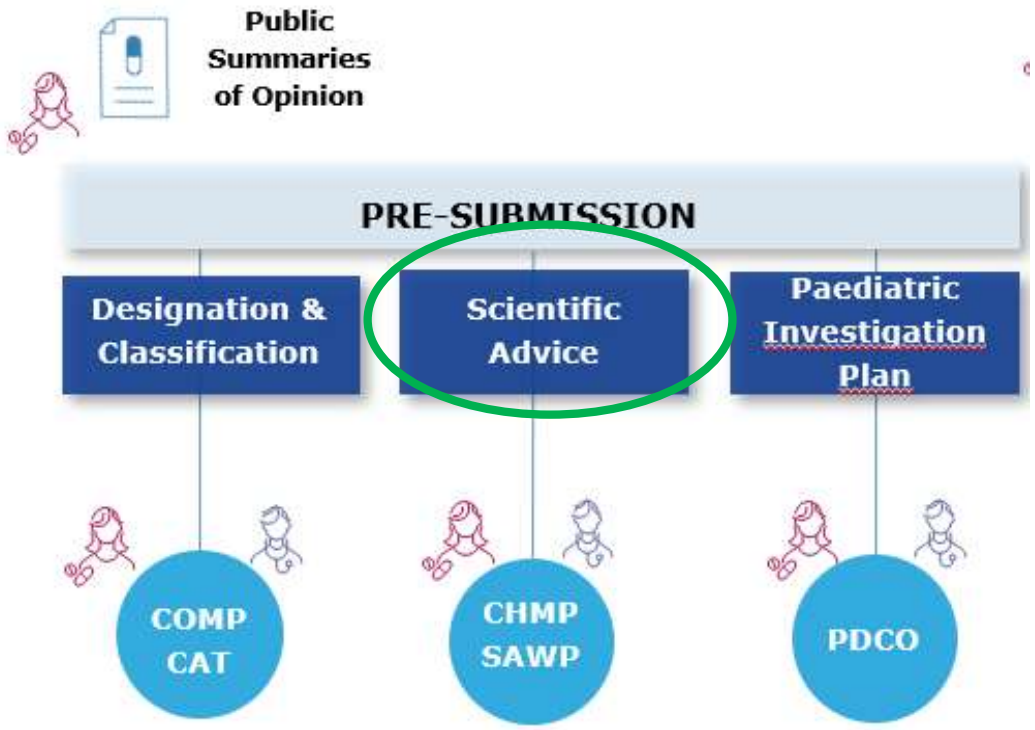
Open Academy

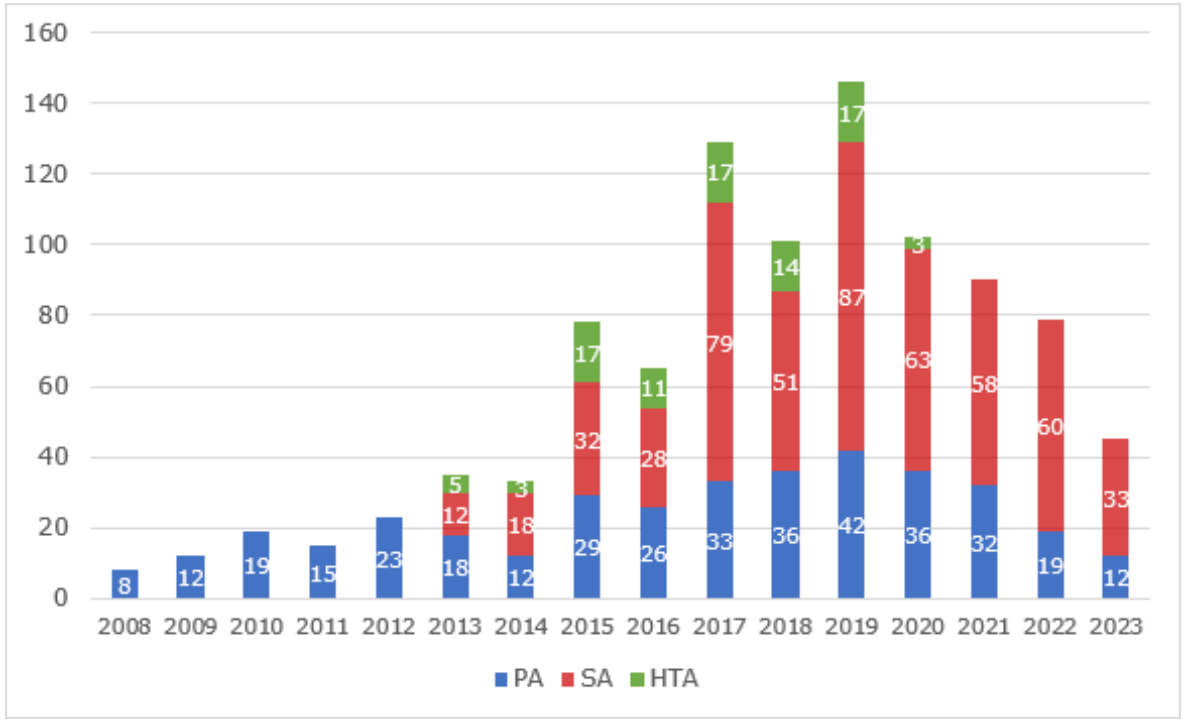
Patient Engagement in pre-submission phase: committees



- Membership in committees: COMP, PDCO and CAT
- Consultations on disease specific issues by committees
- Review of documents destined for public
- Experts invited to scientific advice

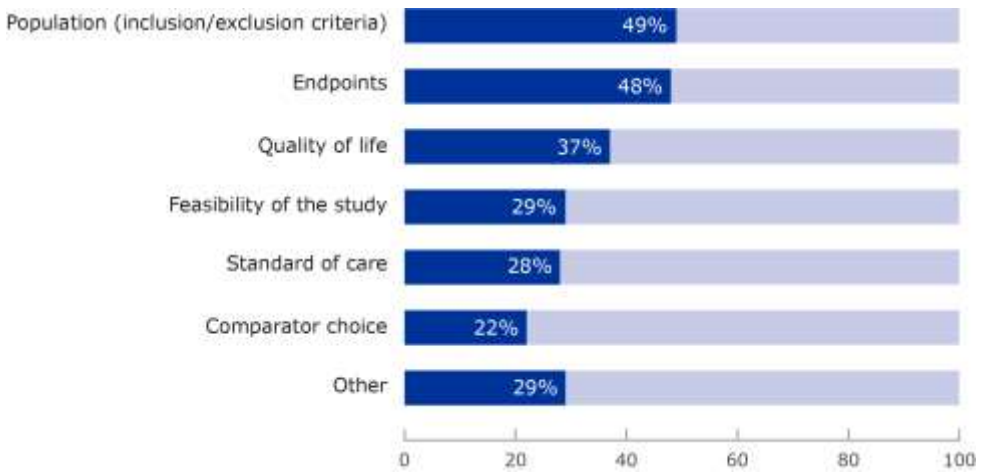
Examples of added value of engagement





Published in [Frontiers in Medicine]

Where patients gave input



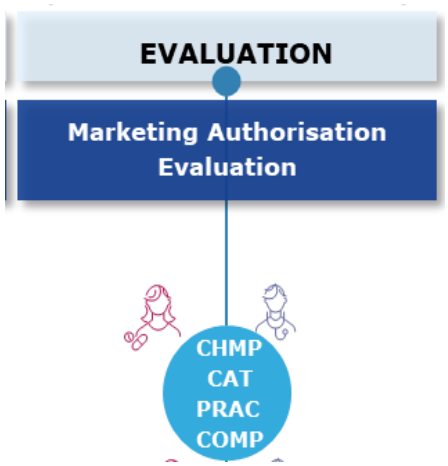
Patient input resulted in further reflection in **52%** of cases.

Added value of patient input and involvement

20% of cases - recommendations made to the developer were modified based on patient contributions.

>85% cases: patient **agreement** with the proposed development plan.

Patient Engagement in evaluation phase: CHMP



EVALUATION

Day 1: Start

Consultation of ORGANISATIONS

Day 80: Assessment Report

Day 120: List of Questions

Day 150: Joint Assessment Report

Day 180: List of Outstanding Issues/ Oral explanation

Day 210: Opinion

Day 277: Commission Decision

Scientific Advisory/ad hoc expert Groups

Oral explanations

Consultation of INDIVIDUALS

Information requested from stakeholders and impact

PATIENT/CARER EXPERIENCE OF:

indication

Please include below any aspects that are of particular importance to patients/carers, such as information on:

- standard treatments and how acceptable they are,
- therapeutic/unmet medical needs,
- quality of life,
- what benefits would be hoped for in new medicines as well as what level of side effects would be considered acceptable,
- considerations for pregnant people/people of child-bearing potential, where applicable.

Also mention any aspects about the condition or its treatment that you feel are not well-understood or not sufficiently considered.

You may include anything else you feel is important for EMA to know. Please try to keep your main points to 1-2 pages; if necessary, include more details in an appendix.

HEALTHCARE PROFESSIONAL EXPERIENCE OF:

indication

Please include below any aspects that are of particular importance to healthcare professionals, such as information on:

- the standard of care or available treatments and to what extent they cover the intended indication;
- the treatment duration; and, if in your view, the duration needs to be optimised;
- any possible therapeutic/unmet medical needs;
- what benefits you would hope for in new medicines; as well as what level of side-effects you would consider manageable for patients;
- considerations for pregnant people/people of child-bearing potential, where applicable.

Please also mention any aspects about the condition or its treatment that you feel are not well-understood or not sufficiently considered.

Please include anything else you feel is important for EMA to know. Please try to keep your main points to 1-2 pages; if necessary, include more details in an appendix.

Information received is reflected in the assessment report under dedicated sections for patients and HCP input

Examples of patient input

Scientific advice

- Rare epilepsy
- Use of comparator

Consultation during evaluation

- Multiple myeloma
- Benefits versus risks

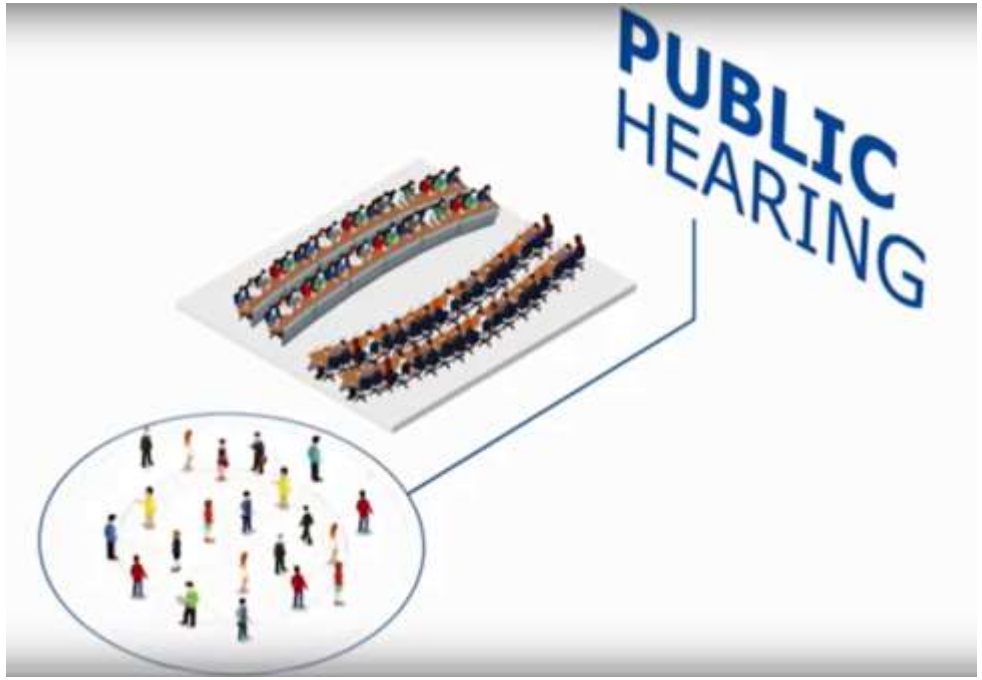
Review of medicine information

- All authorised medicines
- Ensure information is clear

Public hearings at PRAC

2017: Valproate containing medicines

2018: Quinolones and fluoroquinolones



Stakeholder meetings

With patients and healthcare professionals

❖ **Valproate;**

- Written consultation – public hearing – stakeholder meeting – written consultation

❖ **Retinoids;**

- Written consultation – stakeholder meeting

❖ **Methotrexate**

- Written consultation with HCP – medication errors – different doses depending on condition being treated

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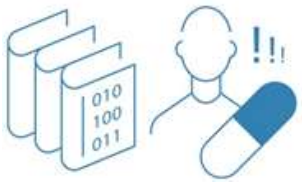
Expanding patient data generation to support medicines development and evaluation



Broaden patient data collection (representing wider patient community):

- Collect patient preferences on acceptable risks vs benefits; on specific treatment options
- Gather patient perspectives; facilitated by patient organisations
- Liaise with other EMA divisions / committees / sponsors for priorities / pipeline / Healthcare professional

Transparency

- Declarations of Interest – publication of declarations and CVs of individual experts
- Eligibility criteria for organisations and publication of funding
- Publication of agendas, minutes, highlights of committees
- Civil society members in committees
- Proactive publication of clinical trial data
- Public Hearings
- Public meetings

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Thank you

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