EMA
EUROPEAN MEDICINES AGENCY

Who can and should represent self-help and patient interests?

Experience of the European Medicines Agency in patient engagement

GÖG-Colloquium | Wer kann und soll Selbsthilfe- und Patienteninteressen vertreten? Erfahrungen der Europäischen Arzneimittel-Agentur EMA.

Maria Mavris (Patient Liaison)
Public and Stakeholder Engagement Department

Content

- European Medicines Agency – what we do
- Journey of patient involvement
- Different types of patient representation at EMA
- Examples of patient input
- Remuneration of experts
- Reporting side effects of medicines
- Conclusion

At EMA, patients are experts like all other experts

Who are the patients?

- People living with conditions
- People caring for patients
- Parents
- Consumers
- Patient representatives
- Members of patient organisations

EMA activities with patients supported by an Engagement Framework

European Medicines Agency

What we do

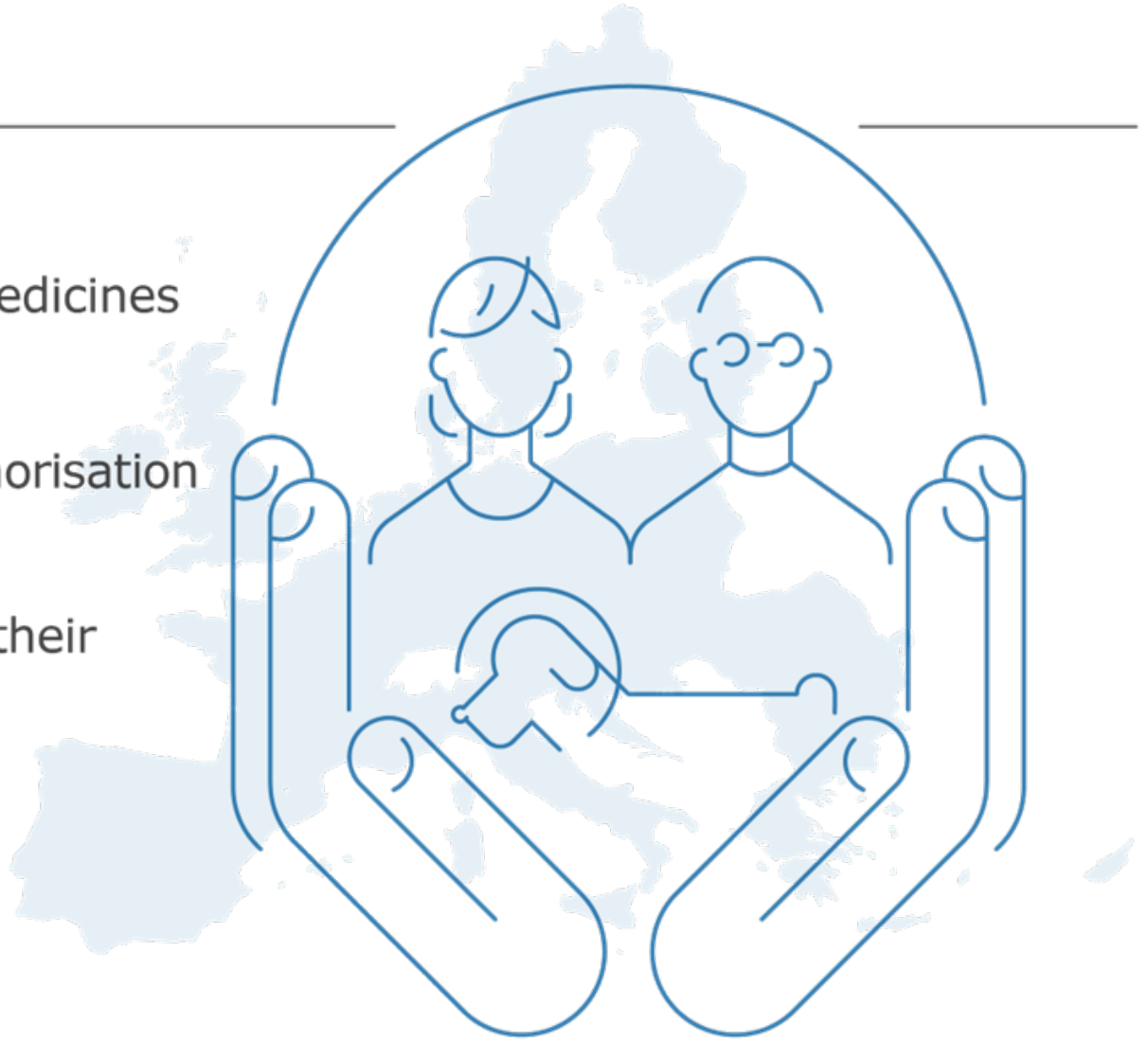
Protect human and animal health

Facilitate development and access to medicines

Evaluate applications for marketing authorisation

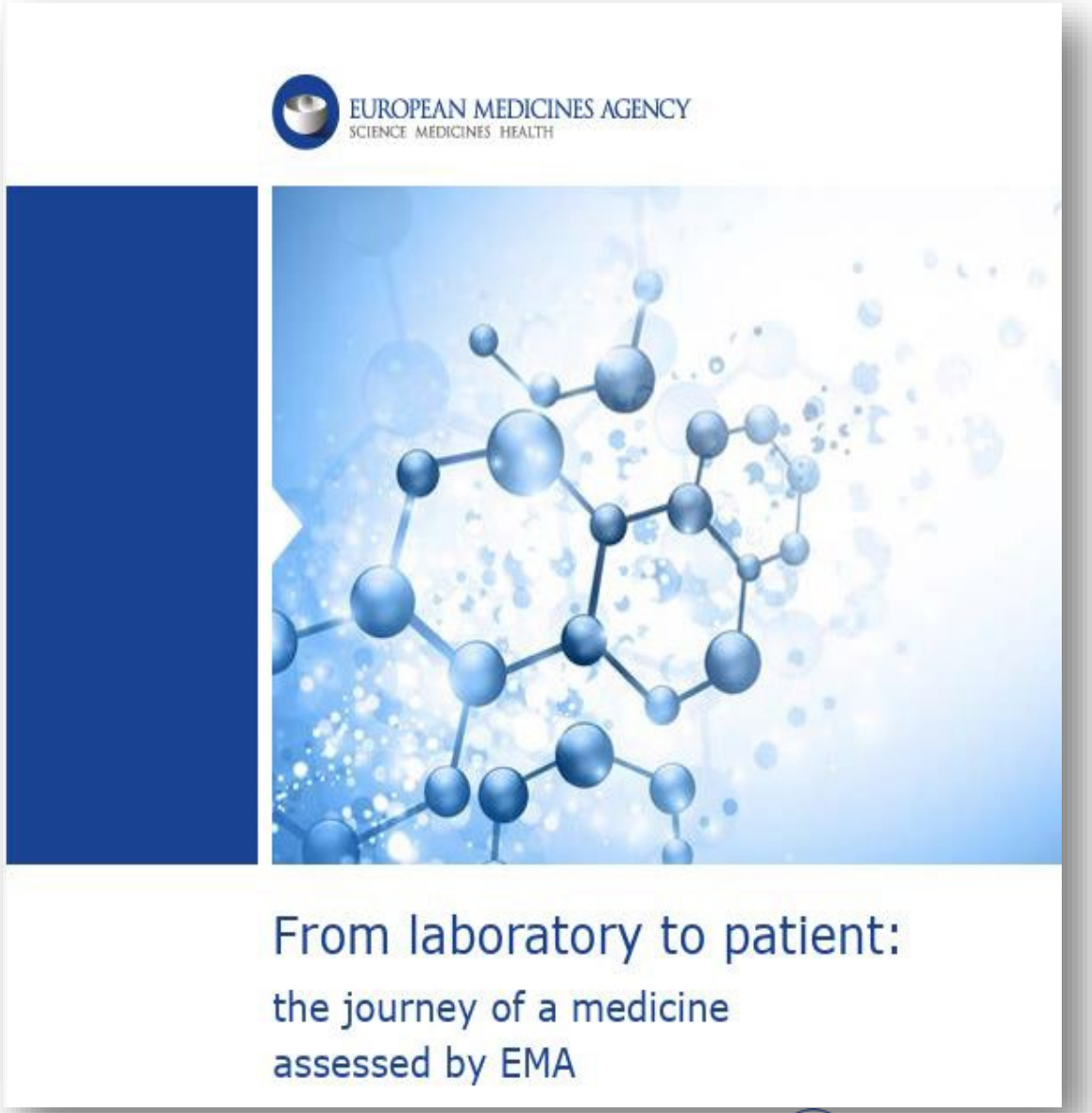
Monitor the safety of medicines across their life cycle

Provide reliable information on human and veterinary medicines to patients and healthcare professionals



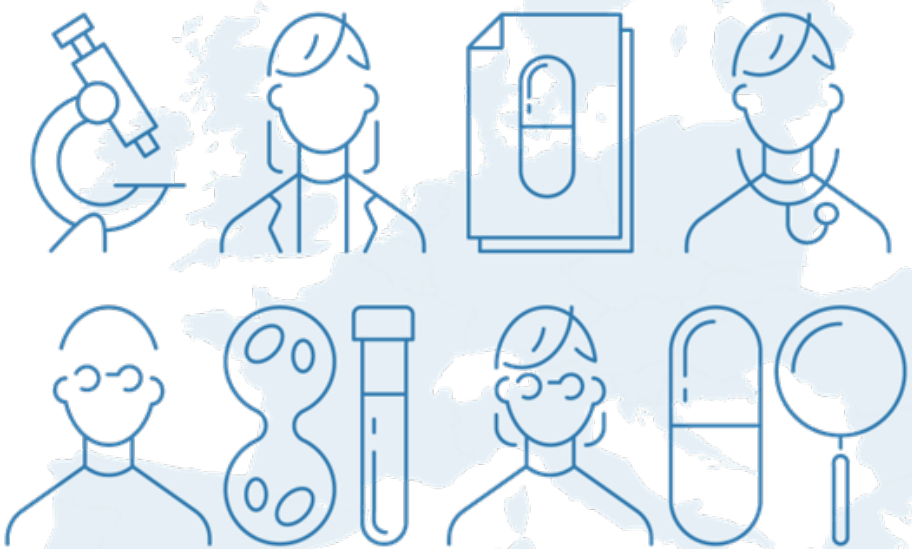
What EMA is not responsible for

- Authorisation of clinical trials
- Pricing or availability of medicines
- Advertising of medicines
- Patents on medicines
- Homoeopathic medicines
- Food supplements and cosmetics
- Develop treatment guidelines or provide medical advice



Who we are

~4000 scientific experts from across Europe



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

1995 EMA established

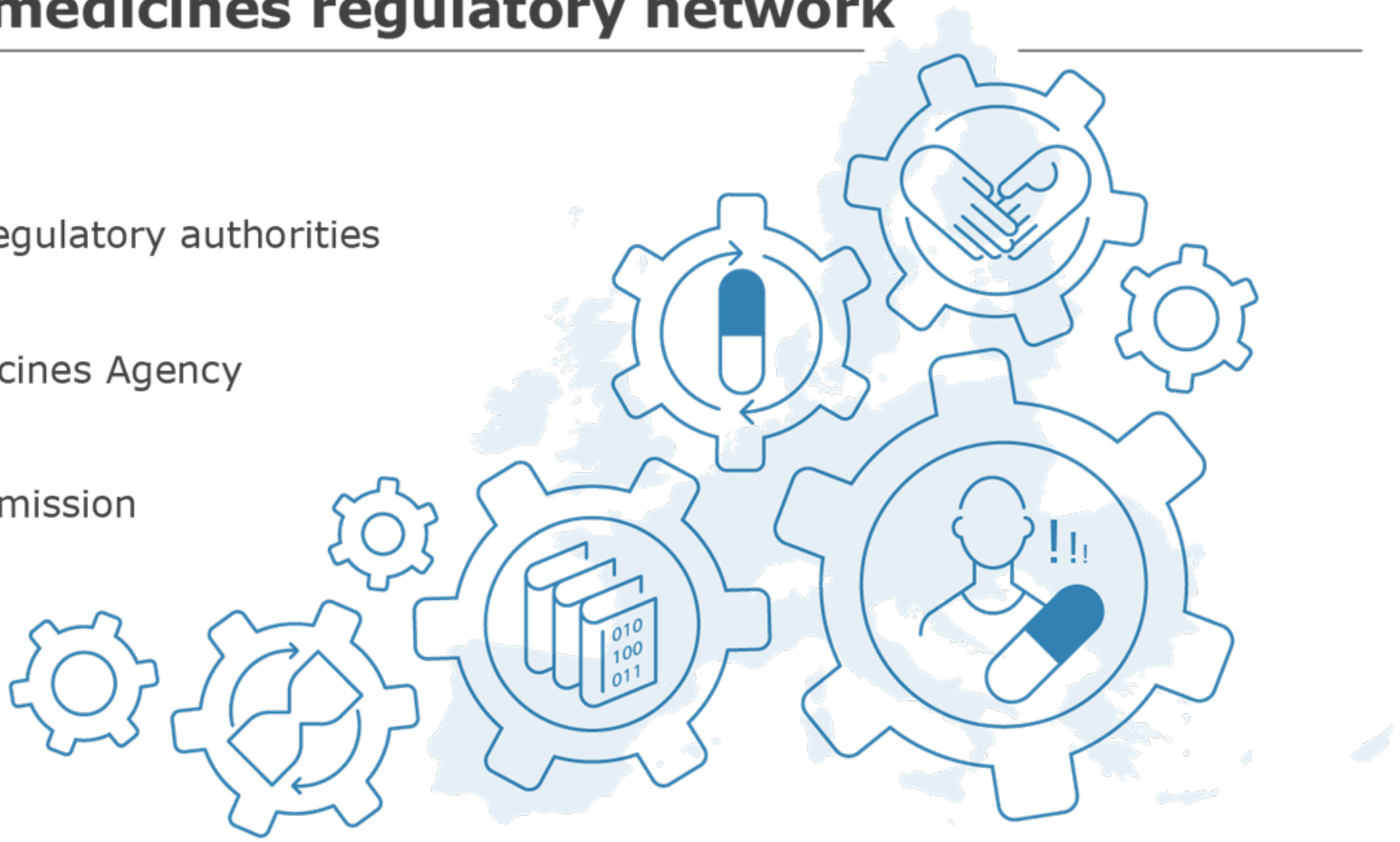
~800 staff members

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

EMA

The European medicines regulatory network

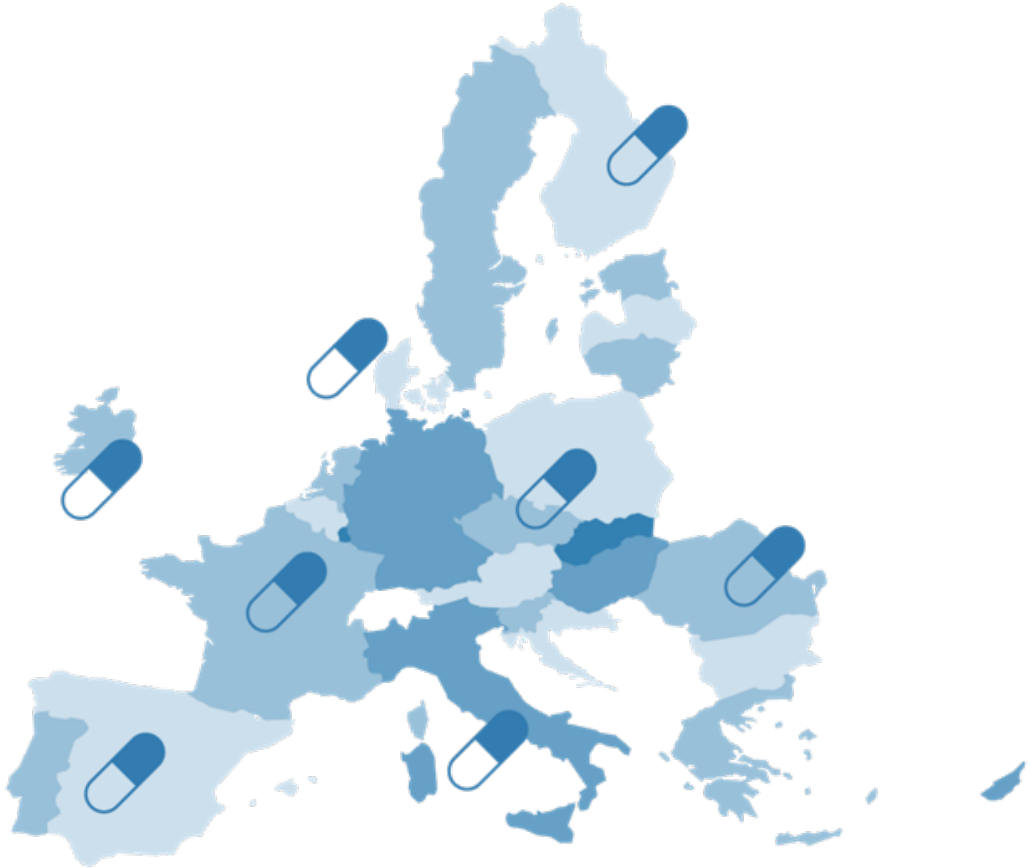
- ~50 national regulatory authorities
- European Medicines Agency
- European Commission



How are medicines approved?

Different authorisation routes: one set of common rules

Centralised procedure (via EMA)



National procedures (via Member States)

EMA

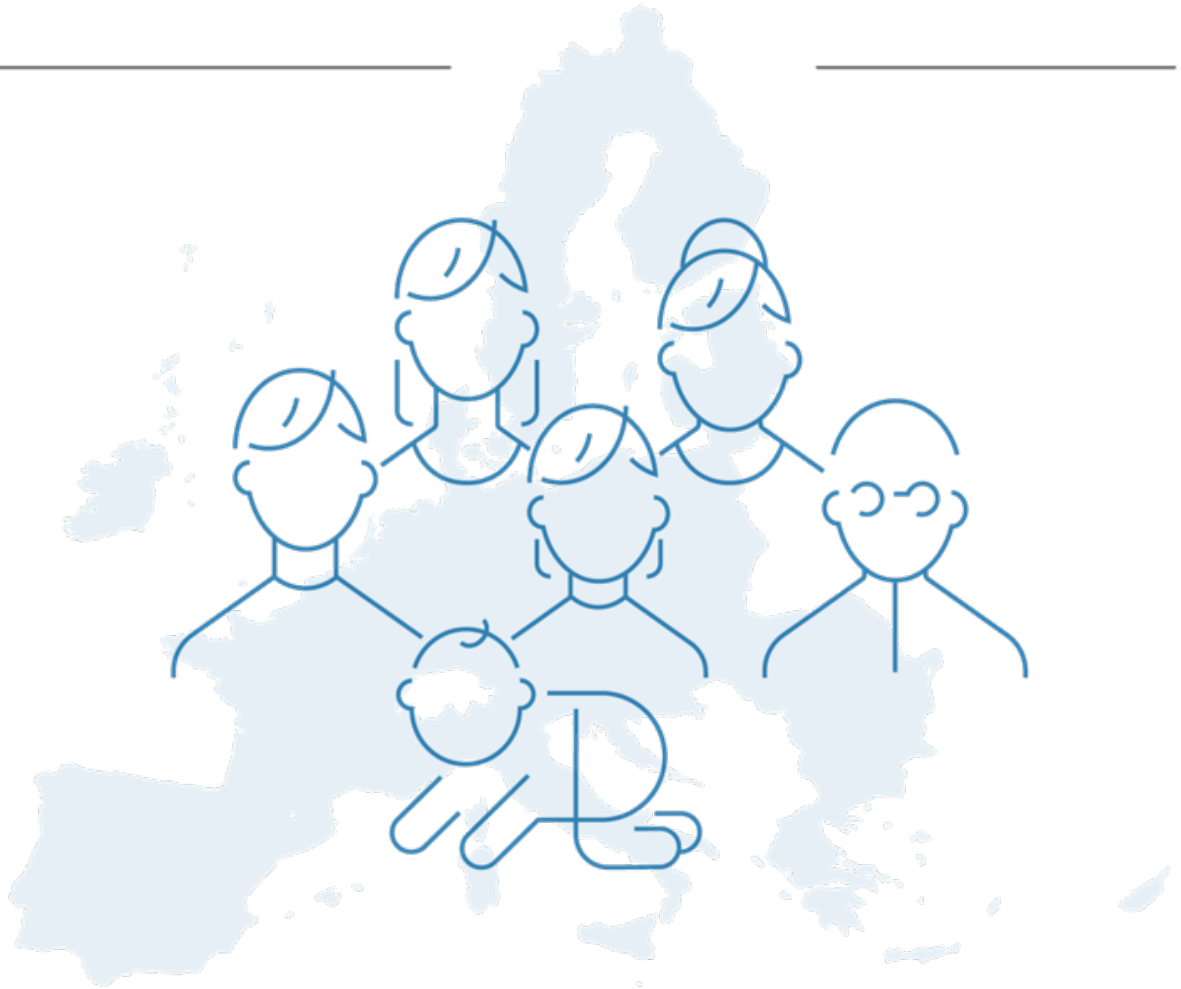
What is the benefit of the centralised procedure for EU citizens?

Medicines are authorised in all EU countries at the same time

Centralised safety monitoring

ABC
XΨΩ
Product information available in all EU languages at the same time

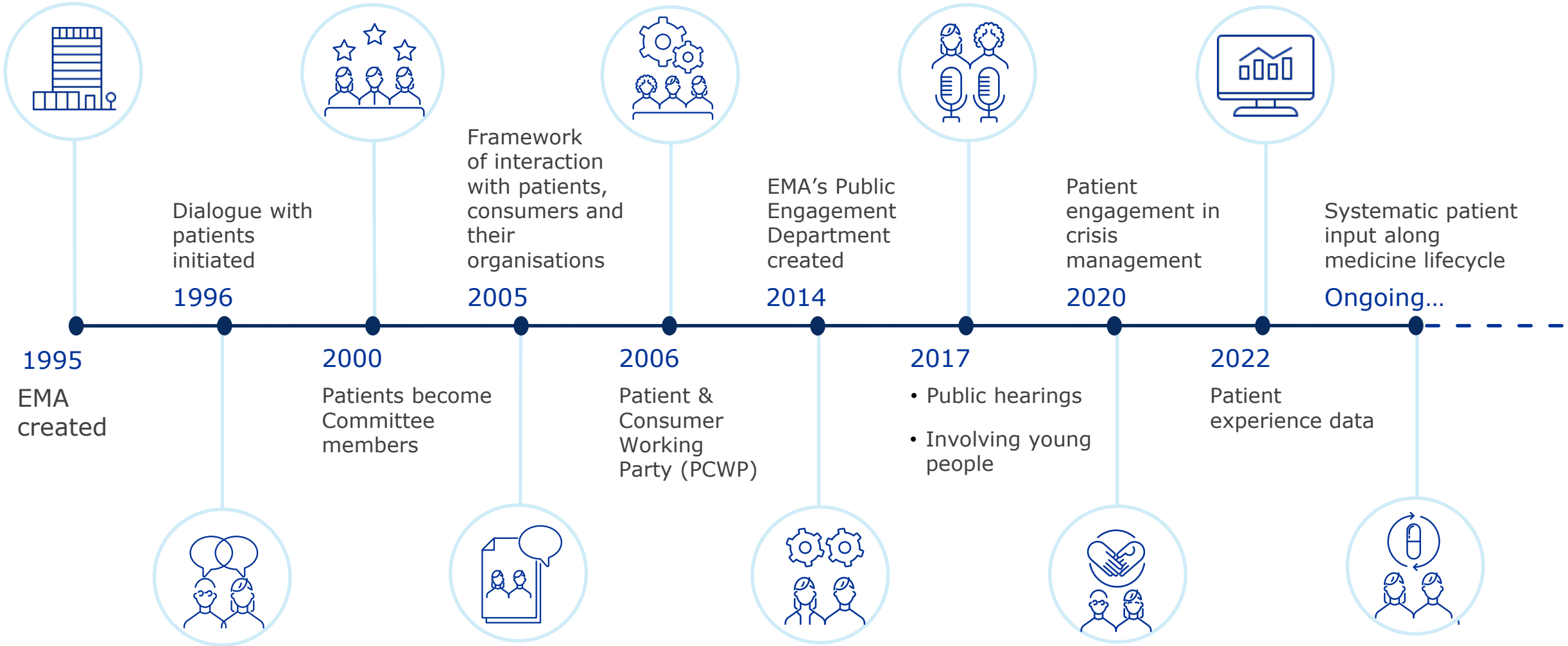
Access to the largest network of experts in medicines regulation



EMA

Patient journey and engagement

Interaction with patients and consumers:



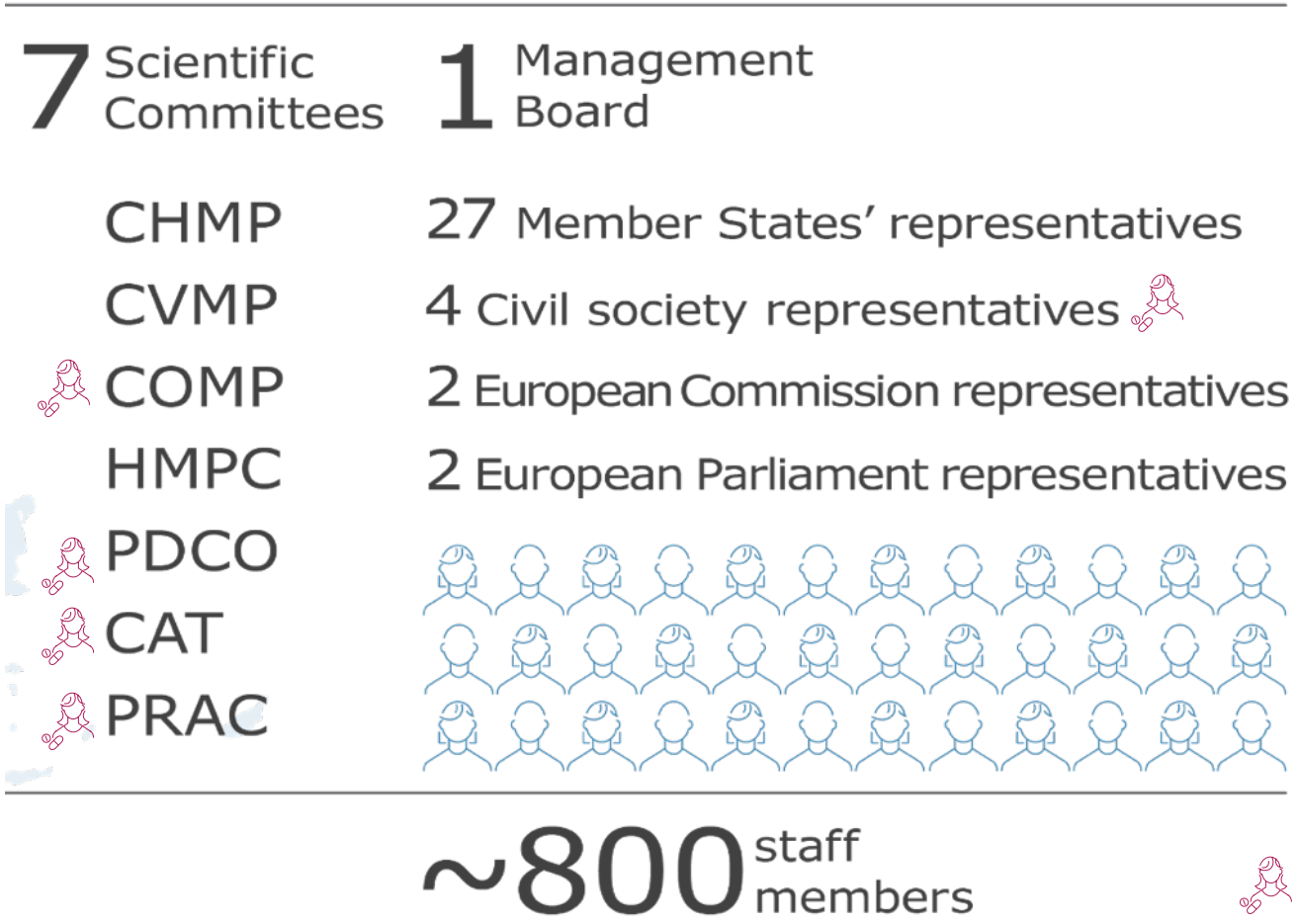
a progressive journey…

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

CHMP – Committee for Human Medicinal Products

CVMP – Committee for Veterinary Medicinal Products

COMP – Committee for Orphan Medicinal Products

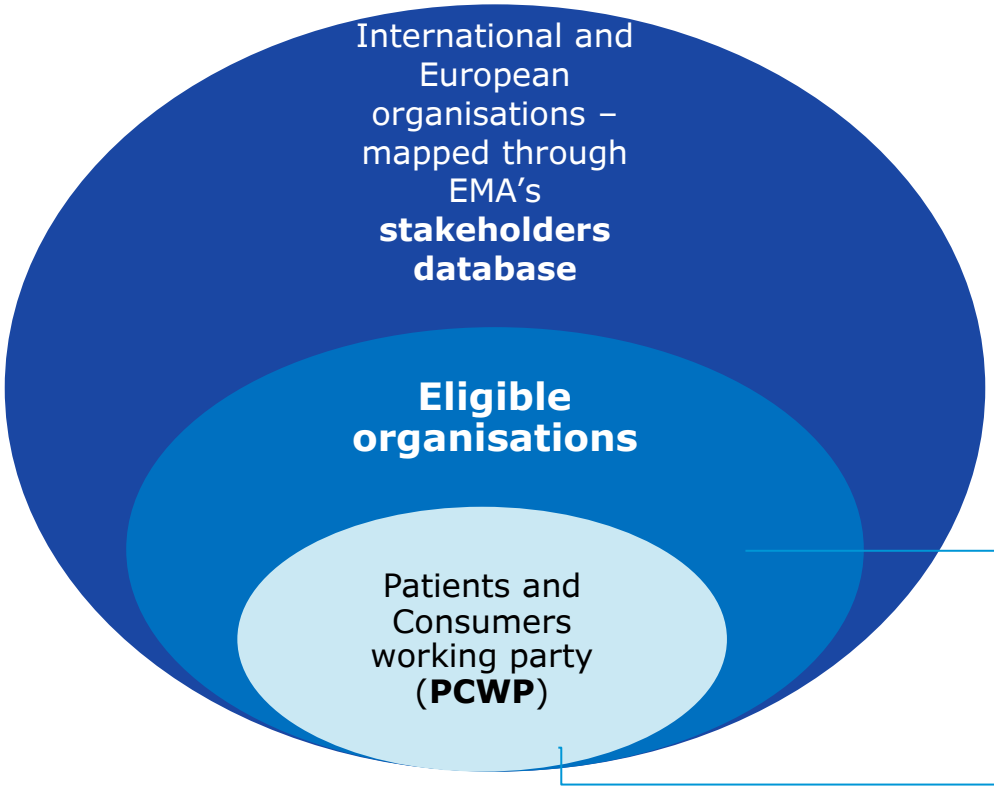
HMPC – Herbal Medicinal Products Committee

PDCO – Paediatric Committee

CAT – Committee for Advanced Therapies

PRAC – Pharmacovigilance and Risk Assessment Committee

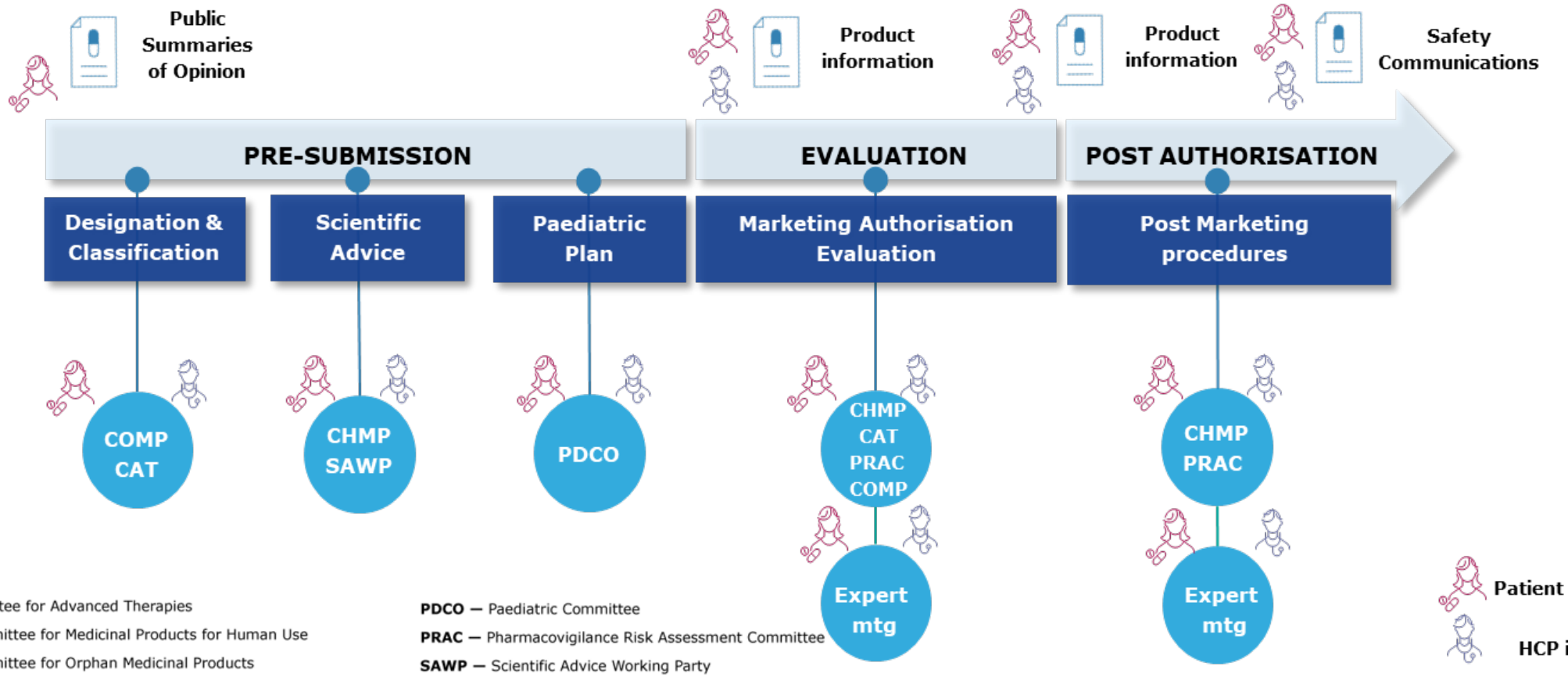
Sources for reaching out to patients



EMA

Bringing expertise into the EU medicines regulatory system

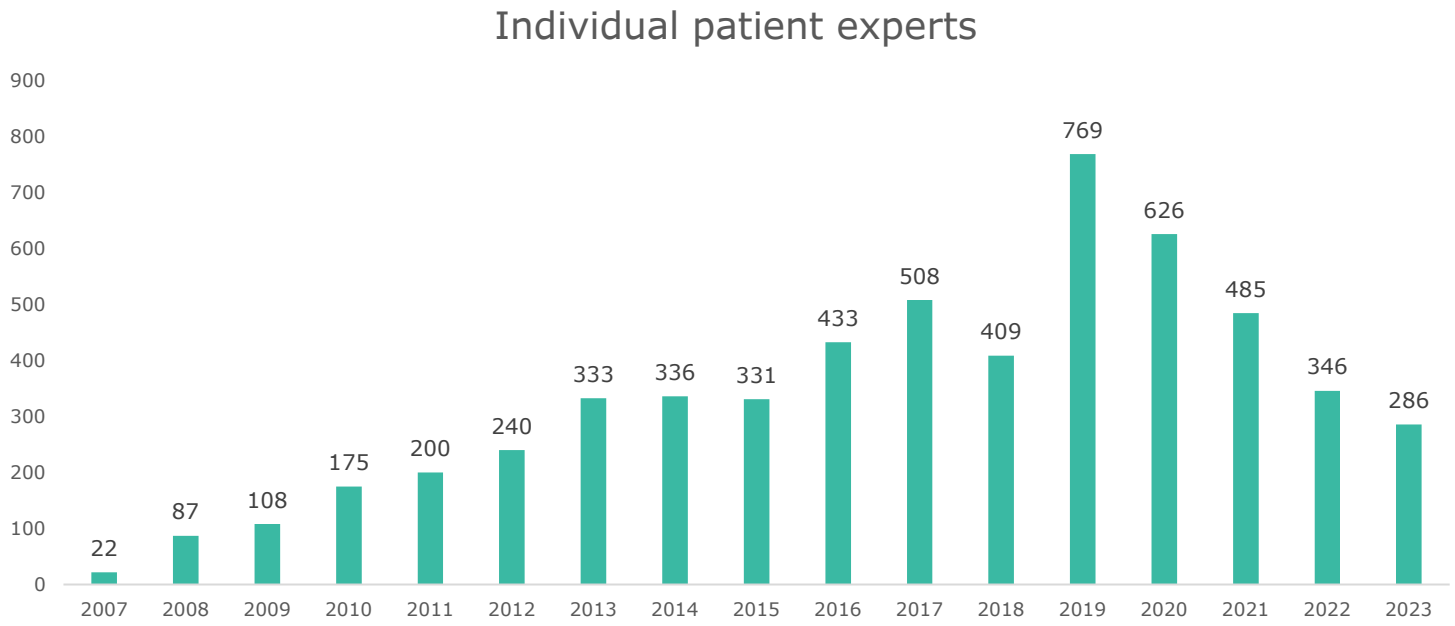
Involvement along the medicine lifecycle at EMA



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

Patients in medicine-specific activities

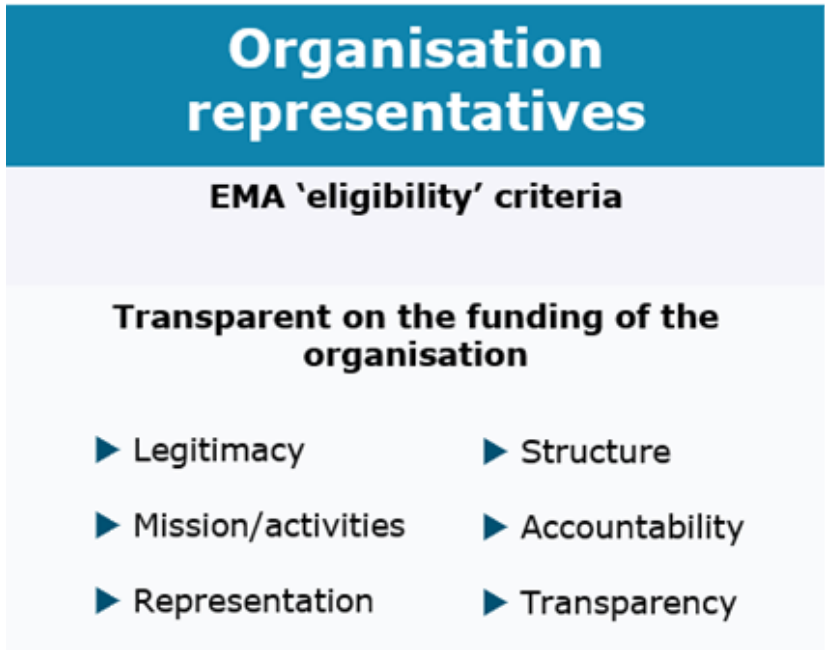


Scientific Advice / Protocol Assistance Procedures
Scientific Advisory/ad hoc expert Groups
Scientific Committee consultations
Review of documents

Representing themselves as individuals

Register as an individual experts

Criteria and transparency



Organisations can become EMA eligible organisations by fulfilling certain criteria.

Individual experts must complete a declaration of interest and confidentiality undertaking

Criteria and transparency

Criteria for involvement of patients:

- Availability
- Ability to contribute in English
- Representation of the appropriate condition
- Conflicts of interests

Organisations can become EMA eligible organisations by fulfilling certain criteria.

Individual experts must complete a declaration of interest and confidentiality undertaking

Engagement and support

One size does not fit all!

Methodologies for engagement

Face to face meeting

Committee - working party- expert meetings

In writing

written responses – reviews - surveys

Training and support

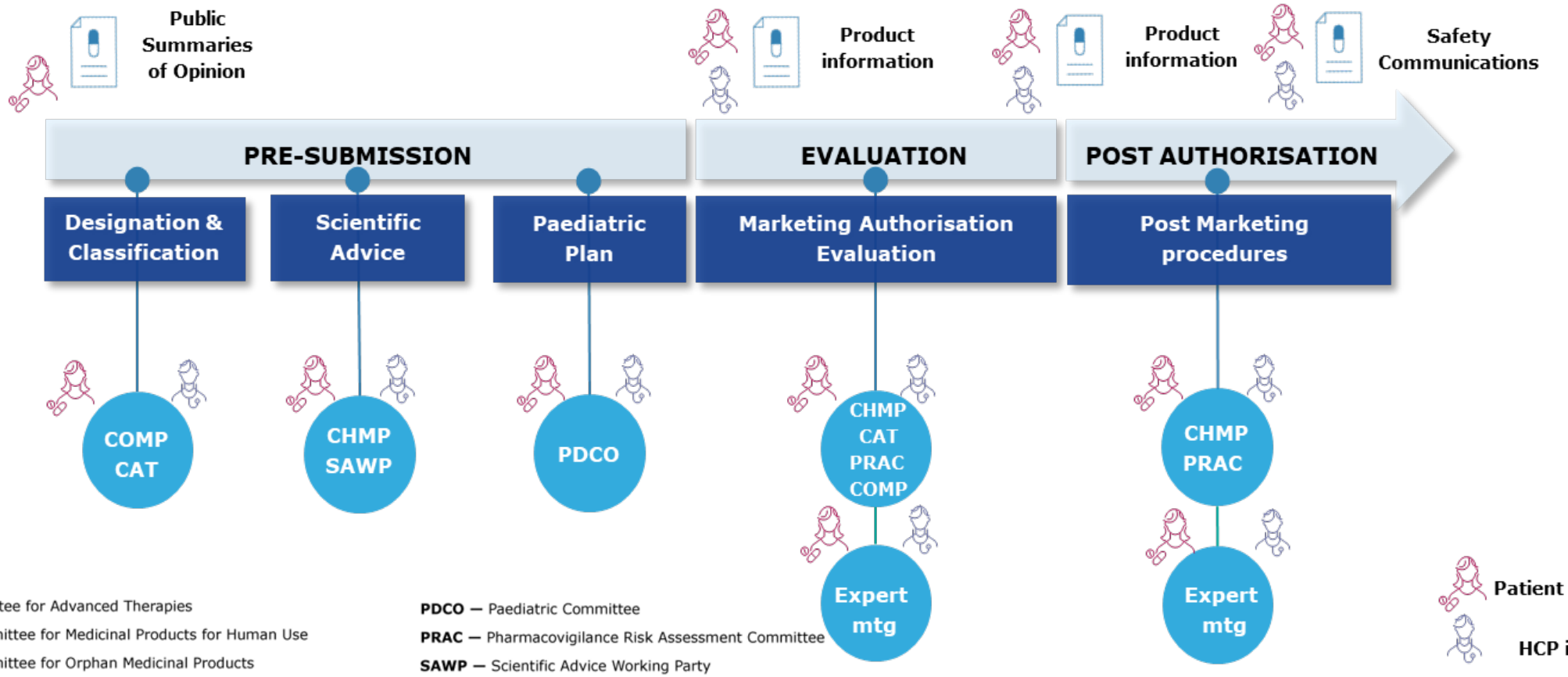
- EMA training day
- Information sheets
- Videos on EMA website
- Information on webpages
- One to one support

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

Bringing expertise into the EU medicines regulatory system

Involvement along the medicine lifecycle at EMA



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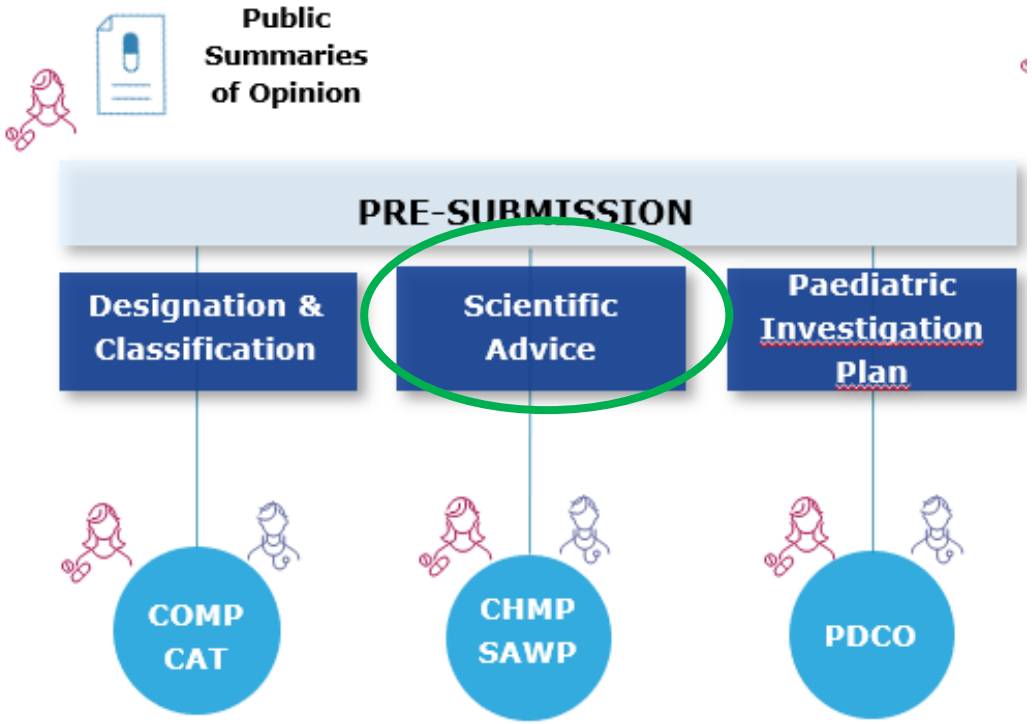
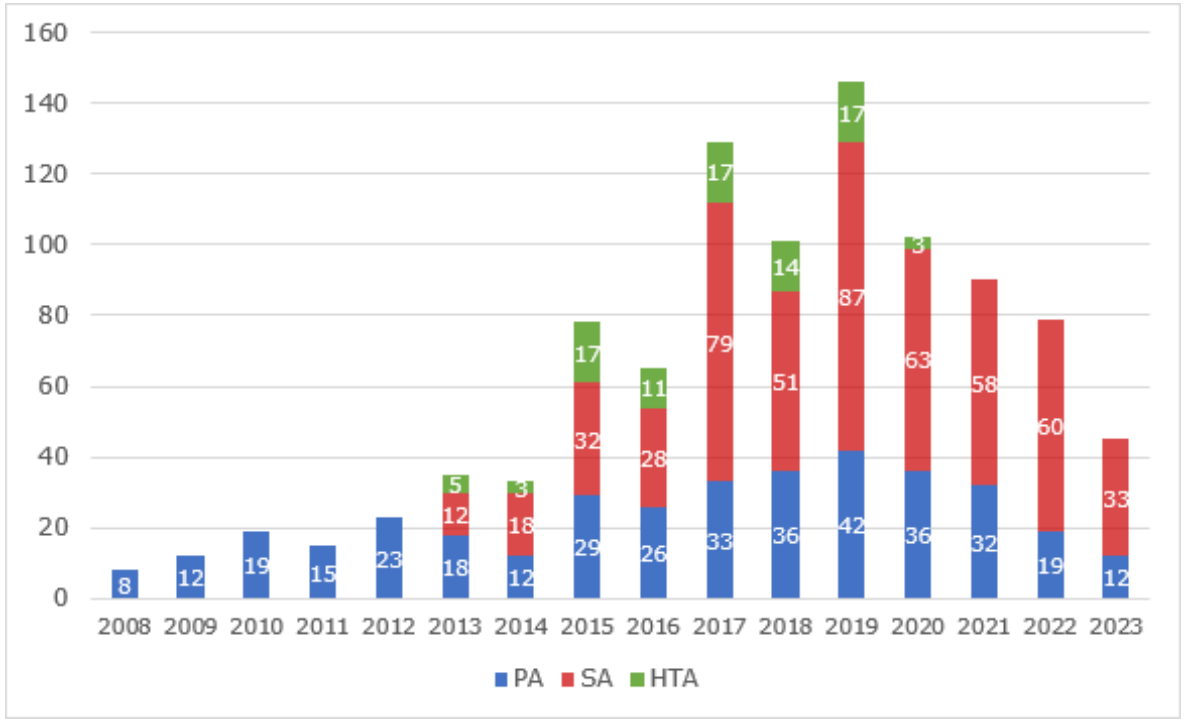
Examples of added value of engagement

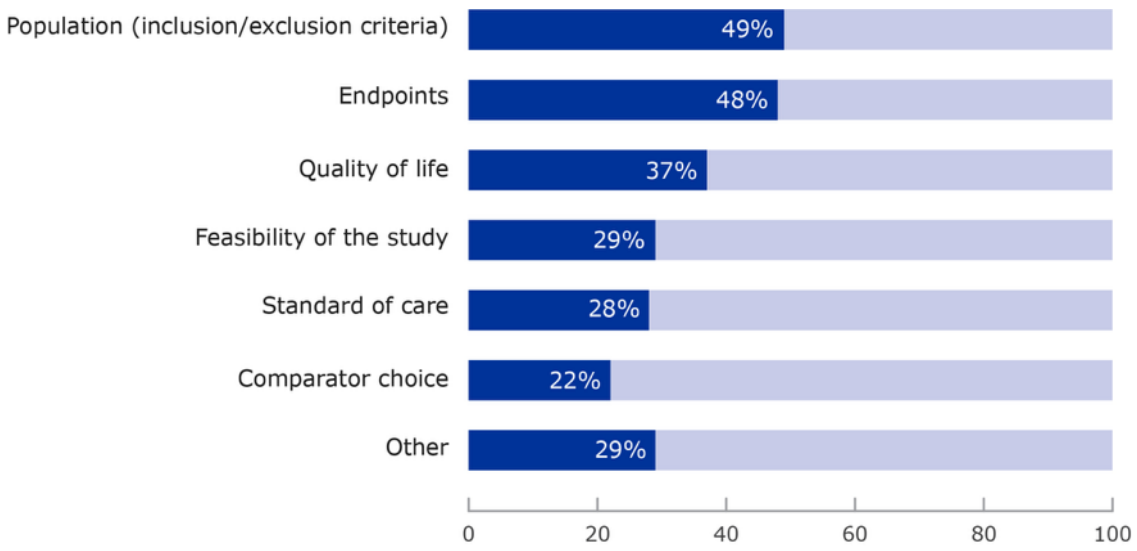




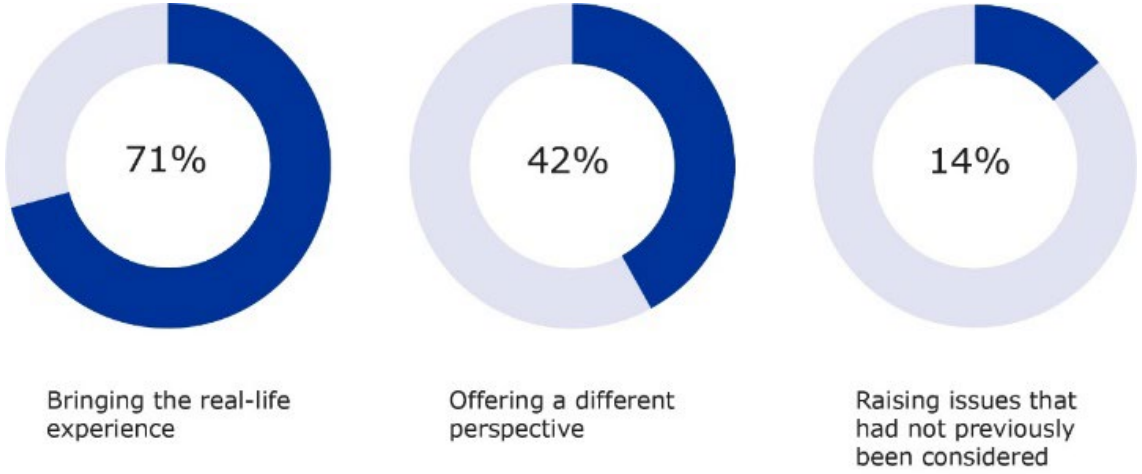
Published in Frontiers in Medicine

PA – protocol assistance, SA – scientific advice, HTA – health technology assessment

Where patients gave input



Added value of patient input and involvement



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

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

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

Patient contributions – some examples

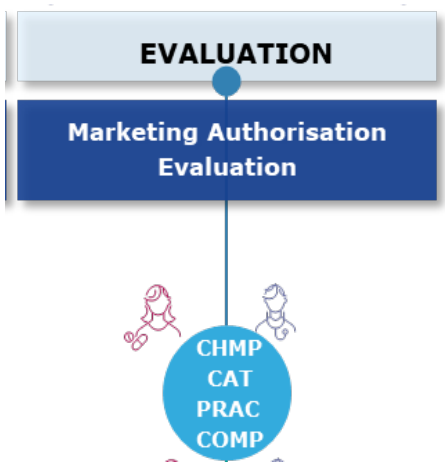
Rare epilepsy

- Comparator medicine
- Patient survey
- Input to Scientific Advice Working Party
- Added to letter sent to company to use comparator medicine

Duchenne muscular dystrophy

- Endpoints
- Parent input to discussion
- Committee for Human Medicinal Products
- “Walking is overrated”

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.

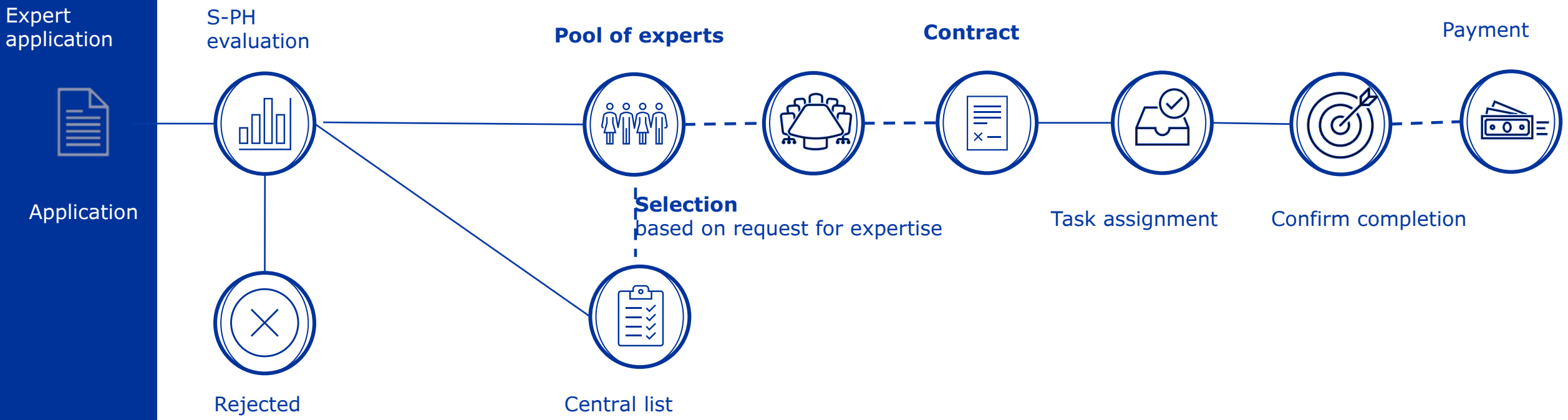
Information received is reflected in the assessment report under dedicated sections for patients and HCP input

Remuneration

Supporting patients and healthcare professionals (HCPs)

- Support participation of patients and HCPs in EMA activities.
- Improved access to input from users of medicines in real life for the optimal scientific outcomes.
- Recognise the value of input from civil society - positive perception among stakeholders.
- Aligned with the spirit of the review of Pharmaceutical legislation.
- Financial support from a regulatory authority will foster independent input from patients and HCPs.

The process of onboarding experts to the pool for remuneration

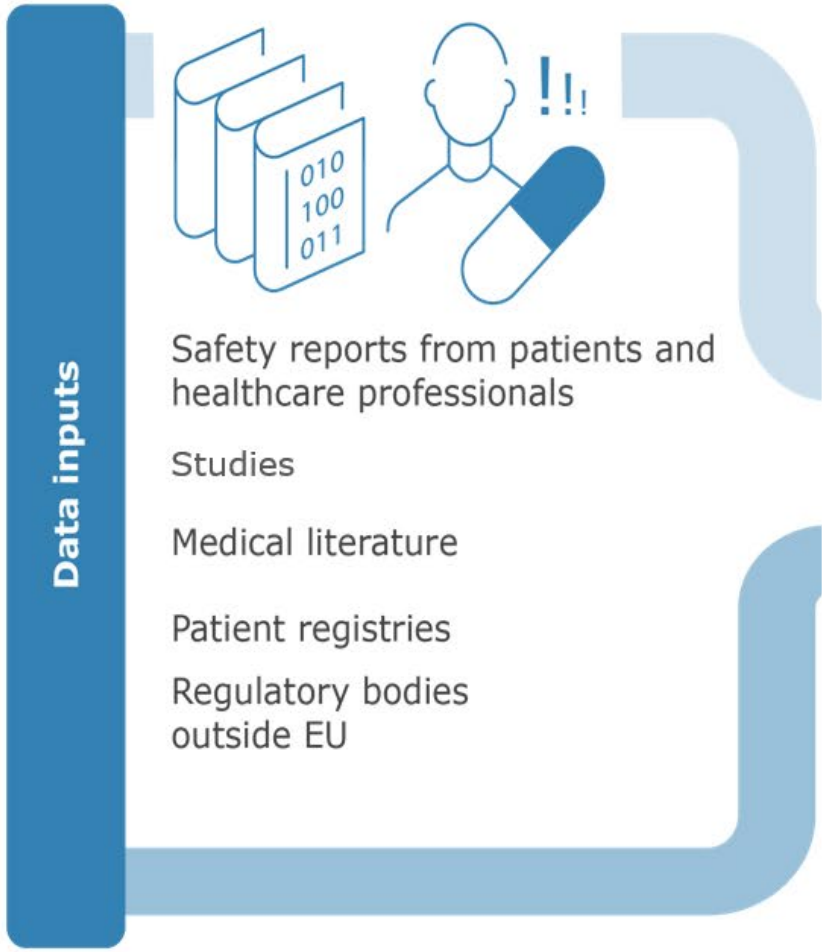


Which activities are covered in the contract?

No	Category	Activity	Renumeration in full day equivalents	Cost per task
1	**Review of document / information**	DHPC Medicine Overview Package leaflet Safety communication Website information (e.g. EVIP)	0.25	€ 112.50
2	**Providing input at EMA meeting**	SAG meeting Ad-hoc expert meeting (AHEG)	1	€ 450.00
3	**Providing input at Scientific Advice**	Written input to SAWP Oral input at SAWP discussion meeting	0.5	€ 225.00
4	**Providing ad-hoc input at the request from CXMP**	Stakeholder meeting Oral explanation Written consultation	0.5	€ 225.00
5	**Regular participation and input at EMA groups** [II]	EMA working party (PCWP/HCPWP) EMA group[I] Meeting with all eligible organisations	0.5	€ 225.00

Safety of medicines and reporting side effects

Monitoring the safety of medicines across their lifecycle



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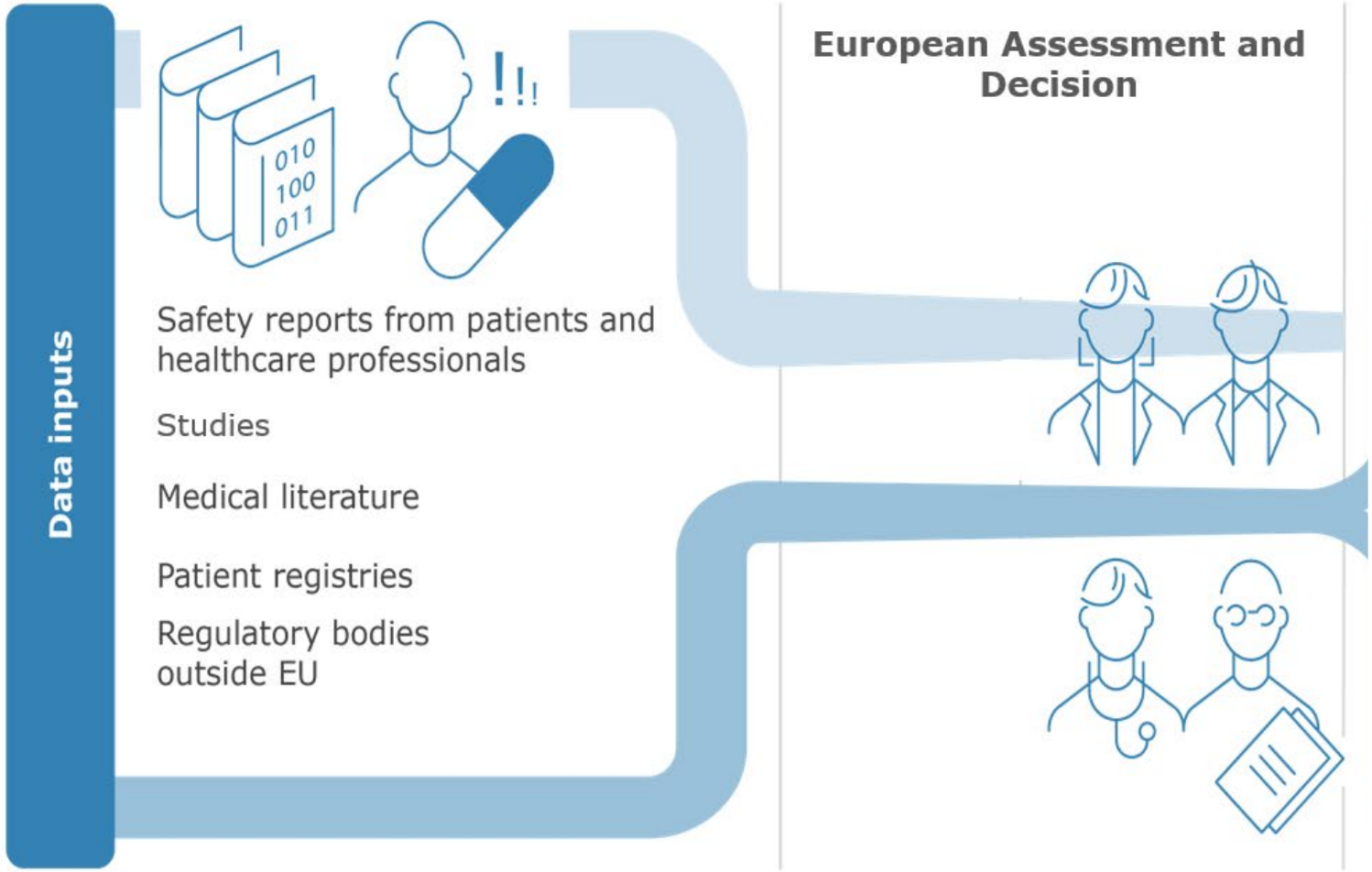

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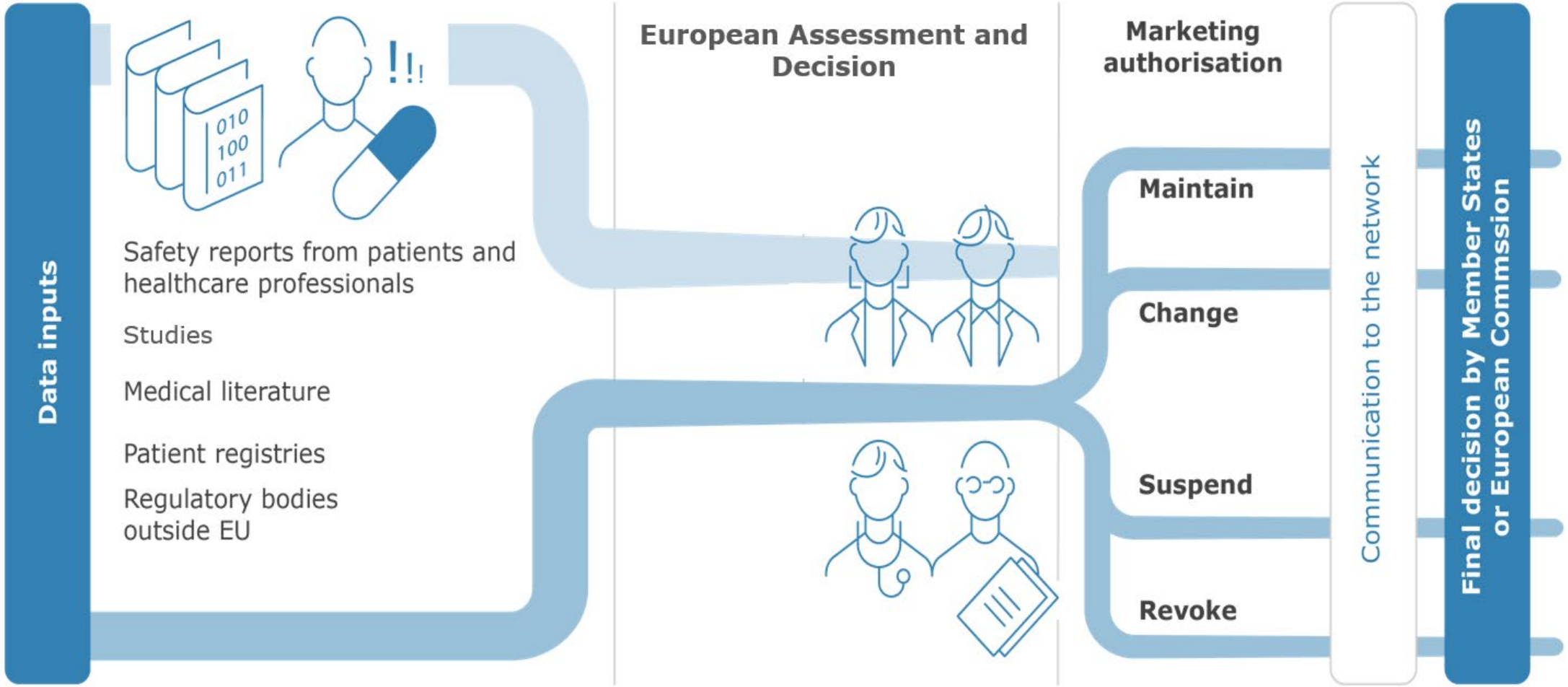
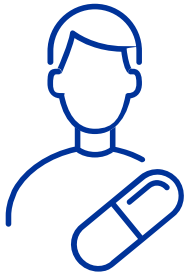


Who can report side effects?

HEALTHCARE PROFESSIONALS



PATIENTS

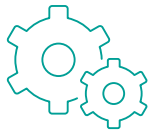
CARERS

By reporting side effects, you can help medicines regulators learn more about the medicine and how it should be used to reduce its side effects.

Did you know?
In Austria, you can report side effects to the Bundesamt für Sicherheit im Gesundheitswesen at www.nebenwirkung.basg.gv.at

Conclusions

- Engaging with patients:
  - Brings **everyday aspects** of living with a disease **into scientific discussions**
  - Helps **bridge the gap** between clinical trial data and real world data
  - Increases **transparency, awareness and understanding: TRUST**
- Engage in a **stepwise approach; learn together** what format works best;
  - **Define roles** - manage expectations
  - Ensure engagement is **mutually beneficial**



Everyone has a role to play to ensure engagement happens

Engaging with patients leads to **more meaningful outcomes** for everyone!

EMA

EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

Thank you

maria.mavris@ema.europa.eu

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