EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

Patient engagement: from a patient centric drug development to authorisation

10th Industry Stakeholder Platform on the Centralised Procedure

27th June 2023

EMA activities with patients supported by Engagement Framework

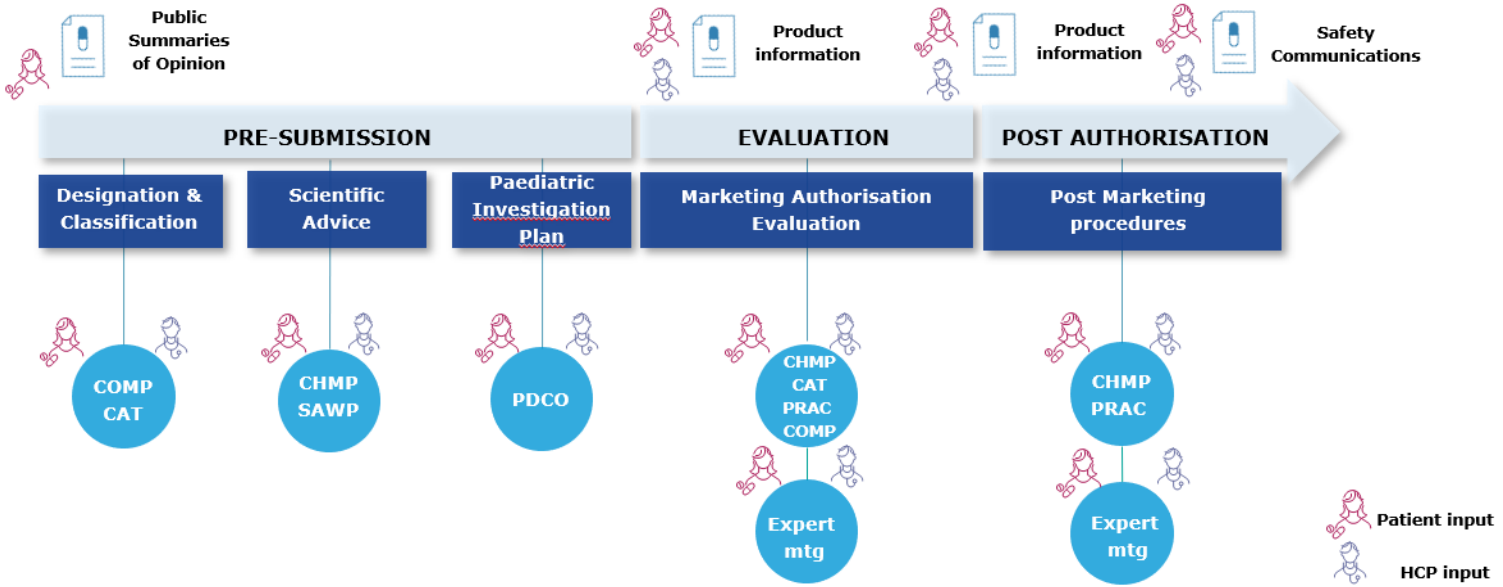
1. A **network** of European patients' and consumers' **organisations**;
2. Patients' and Consumers' Working Party (**PCWP**);
3. A pool of **individual** patients, consumers or carers,
4. **Capacity-building** and training
5. Range of **engagement methodologies** enabling patients and consumers to be 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**.

1 Patient engagement: from a patient centric drug development to authorisation

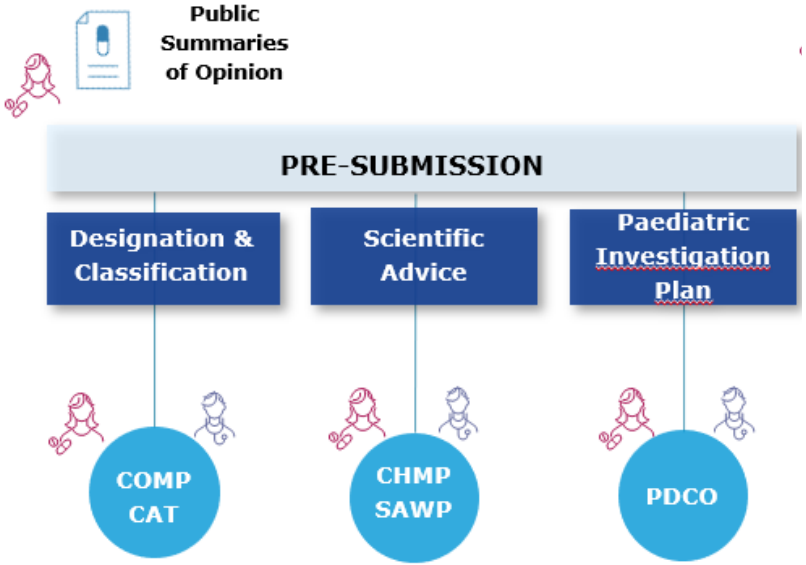
Three categories of patient participation:

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

Stakeholder engagement across medicines lifecycle

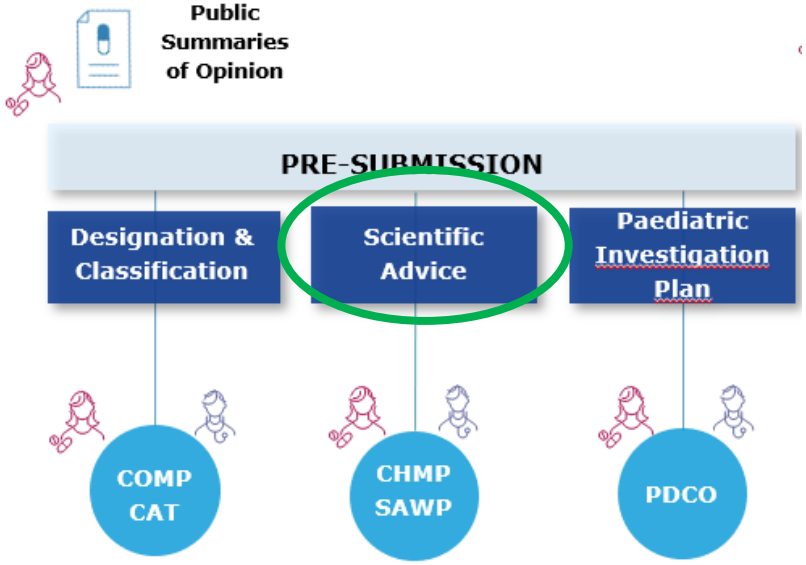


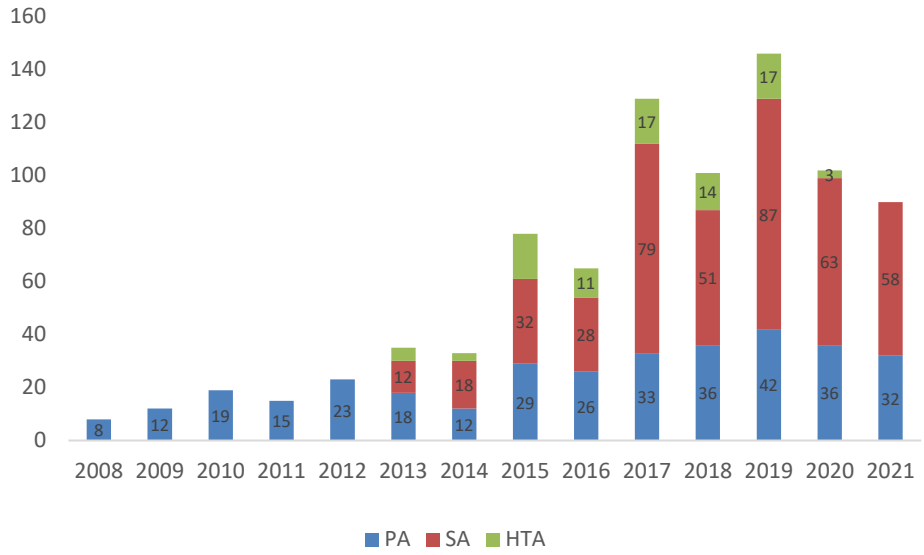
Patient Engagement in pre-submission phase: committees



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

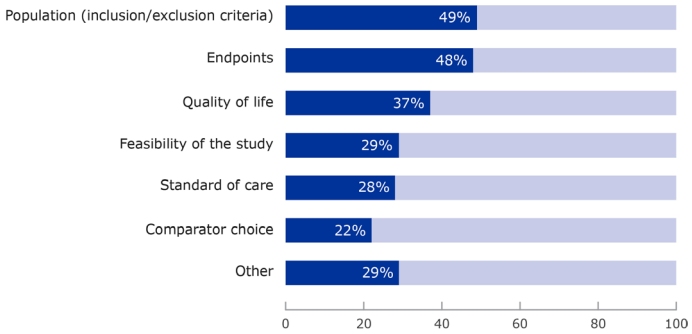
Patient Engagement in pre-submission phase: Scientific Advice



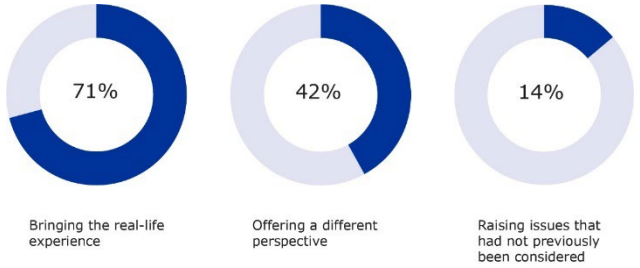


Published in [Frontiers in Medicine]

Where patients gave input



Added value of patient input and involvement

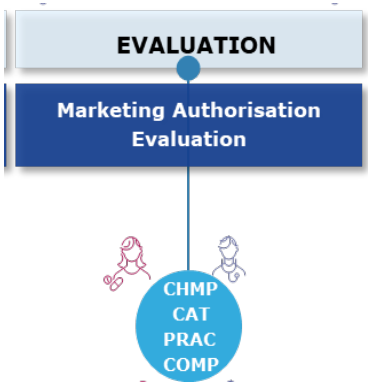


Patient input resulted in further reflection in **52%** of cases.

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

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

Patient 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

Pilot outcome summary

- 37 procedures over 17 months
- Rapporteurs were positive and input received reflected usefulness and benefit of reaching out to patient organisations at start of assessment of MAA's.
- Patients provided new insights that contributed to the D80 assessment report.
- 41% of cases contributed to the development of the first assessment report
- Information from patients related to *daily impacts*, *treatment options*, *perspectives and perceptions of adverse effects*, *what constitutes important improvements and desired benefits for new treatments* have proven to be insightful / helpful
- Pilot now a new methodology to be continued and extended to medicines of potential significant impact.

Next steps

Extension to:

- non-orphan medicines (started September 2022)
- Healthcare professional organisations (started May 2022)
  - Webinar held with patients and HCP organisations 19 April
  - Guide with Q&A in process of development

Exploring how to reference in assessment report – feedback from CHMP to PCWP and HCPWP

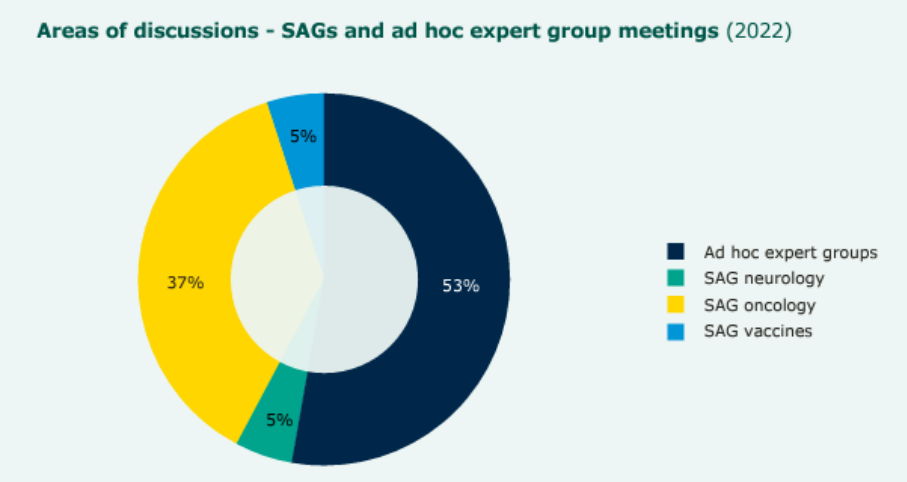
Post pilot: early contact with patients (since 2022)

Month/year	Type of procedure	# responses from PCO
September 2022	3 orphans	1 response
October 2022	3 orphans 2 non orphans	4 responses
1 December 2022	3 orphans 2 non orphans	3 responses and 1 use of previous response for same indication
28 December 2022	1 orphan 1 non orphan	2 responses
26 January 2023	3 orphans 3 non orphans	6 responses
23 February	2 non orphans	2 responses
23 March	3 orphans	2 responses

Scientific Advisory Groups (SAGs)/ ad hoc expert groups

EMA's scientific committees can consult additional experts, patients and healthcare professionals to enrich their scientific assessment of medicines.

These external parties may be involved in SAGs or Ad-hoc expert groups.



33 patients were involved in SAG meetings in 2022

Patient Engagement in CHMP: Oral explanations

Year	N Patients	N procedures	Procedures
2020	10	6	Hopeveus, Dapavirine, Arikayce, Gamifant, Fintepla, Sogroya
2021	7	5	Evrysdi, Zolgensma, Ozawade, Raylumis, Tecentriq
2022	4	2	Miplyffa, Sohonos
2023	2	2	Albrioza, Adakveo

Take home messages

- EMA is constantly exploring additional engagement methodologies
- Support provided to civil society members in CXMP and Management Board
- Training and support with new tools and content
- Multi-stakeholder dialogue is key

Any questions?

Public Engagement Department

www.ema.europa.eu

European Medicines Agency

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