EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

Patient Engagement in Scientific Advice

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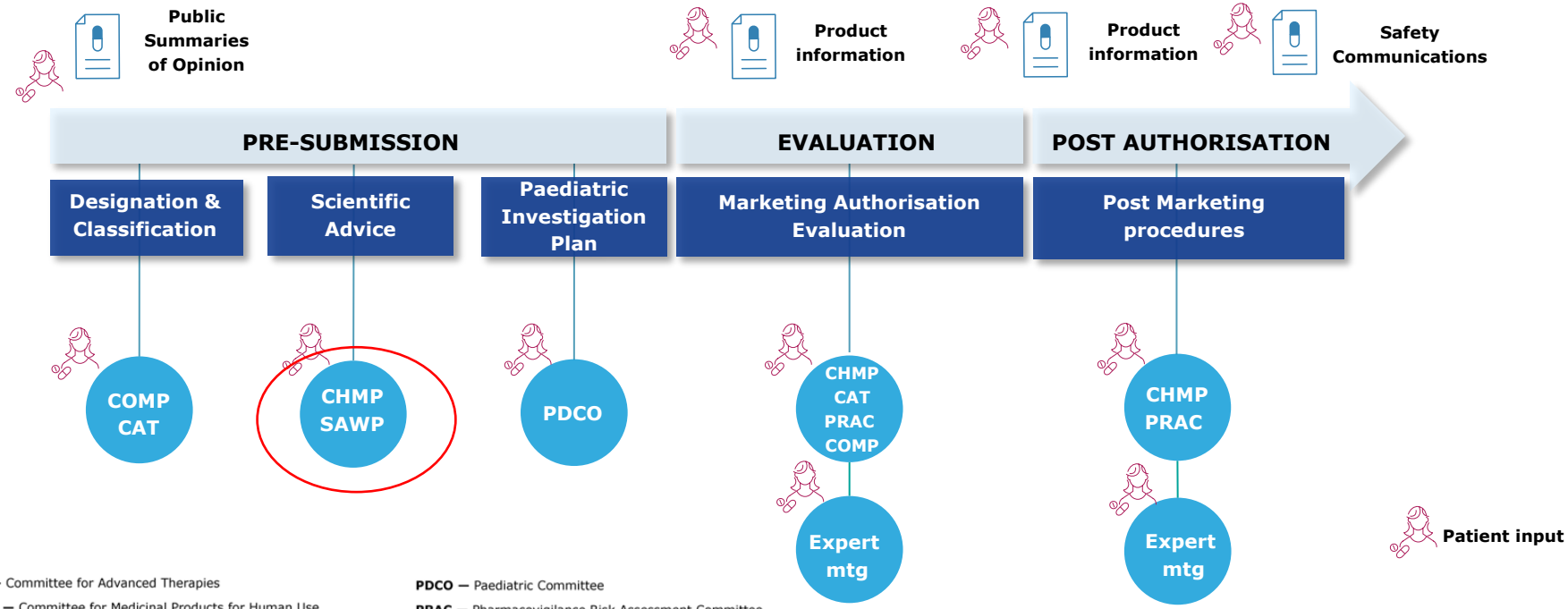
Patients' involvement in EMA regulatory activities



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

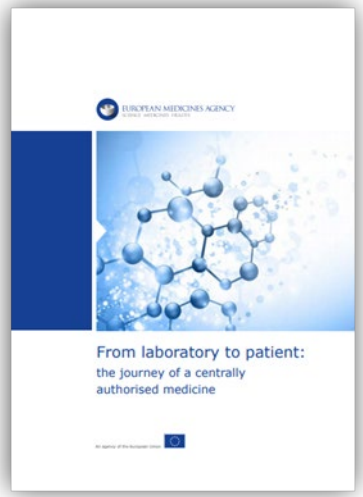
Overview of patient engagement in Scientific Advice

What is Scientific Advice (SA) / Protocol assistance (PA)

The Scientific Advice Working Party (SAWP) answer specific questions from the developers

Scientific advice:

- Requested to EMA at any stage of development
- Advice based on documentation provided by developers
- Recommendations on development for marketing authorisation
- Questions ranging from quality, non-clinical to **clinical**, regulatory, significant benefit (orphan medicines)



Patients participate as **individual experts** – read the documents, ask questions, contribute comments in writing or/and in a meeting

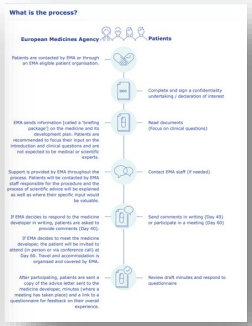
Patients submit a DOI and are bound by confidentiality

How do we find and include patients in SA/PA procedures?

- Eligible organisations
- Previously participated
- Database of individual experts
- Sources on internet for other diseases/conditions

Selection based on:

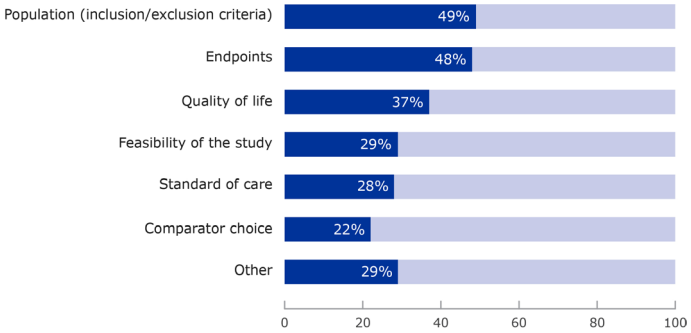
- Experience with disease as patient or carer
- Ability to speak/read English
- Free from conflicts of interest
- Level of experience with medicines development may vary



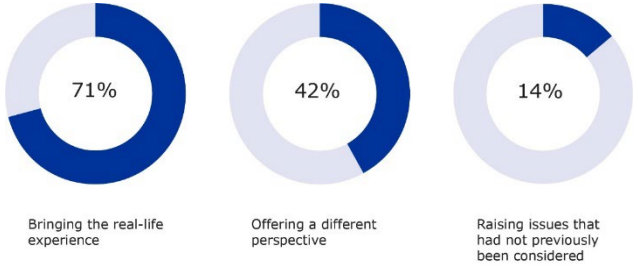
Overview of patient engagement in Scientific Advice

Impact and added value of patients in SA

Where patients gave input



Added value of patient input and involvement



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

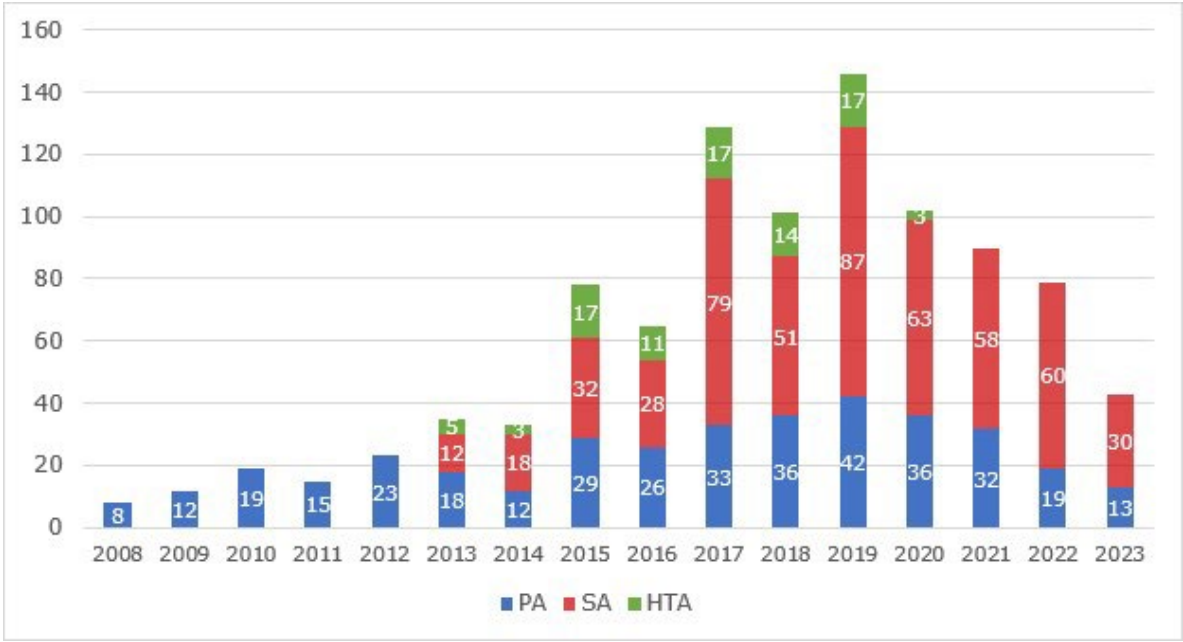
20% of cases - recommendations made to the developer were modified based on patient contributions.

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

The Added Value of Patient Engagement in Early Dialogue at EMA: Scientific Advice as a Case Study. *Front Med*, 20 January 2022

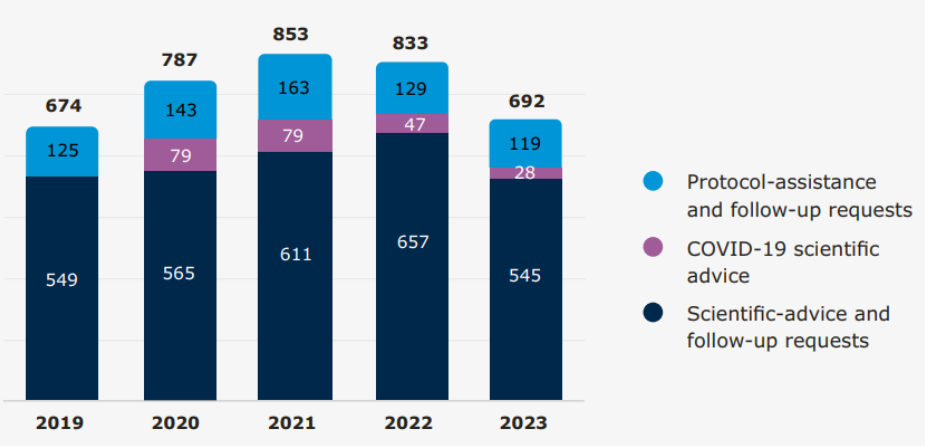
Patient involvement in SA evolution: 2008–2023

- Data for patient involvement since 2008
- First patients in PA – extended to non-orphans in 2013
- Involvement of patients in HTA in 2013



What lies behind the trend

- Reduction in patient numbers explained by various factors
  - Fewer SA requests in 2023
  - Peak for some years linked to EU funding (orphans)
  - Reduced capacity of network to manage requests, with delays in SA procedures
  - Changes in S-PH and SA teams' composition/responsibilities
  - Persisting perception that patient involvement involves extra work



Enhancing patient involvement in the process

- S-PH has been engaged in discussions with SA secretariat to explore ways to improve patient involvement in SA
- Discussions on creation of dedicated section in SA briefing document template
  - Provide a lay summary
  - Patient input included in specific section → reflected in joint report (based on same template)
  - Industry would write → need to ensure content would not be biased
- Ongoing reflections around alignment with EMA initiative on patient experience data (PED): earlier flagging of need to include PED in development plans of companies
  - 2024: Reflection paper, work on CHMP assessment report template

Looking forward

- Continued feedback survey to patients and Scientific Officers, including suggestions for improvement of the process
- Ongoing efforts to reach out more widely to patient communities in areas where there are challenges
- Remuneration of experts: call to be launched for registration in "pool of experts" similar to individual stakeholder database but with eligibility criteria etc. → ready for involvement in scientific procedures and remunerated
- Annual training for patients and healthcare professionals – 5/11/13 December (virtual) with sessions on SA, competing interests, document reviews → eligible organisations can put forward 1 person for the training
- HTA: implementation of Joint Clinical Assessments & patient involvement in regulatory and HTA evaluations

Any questions?