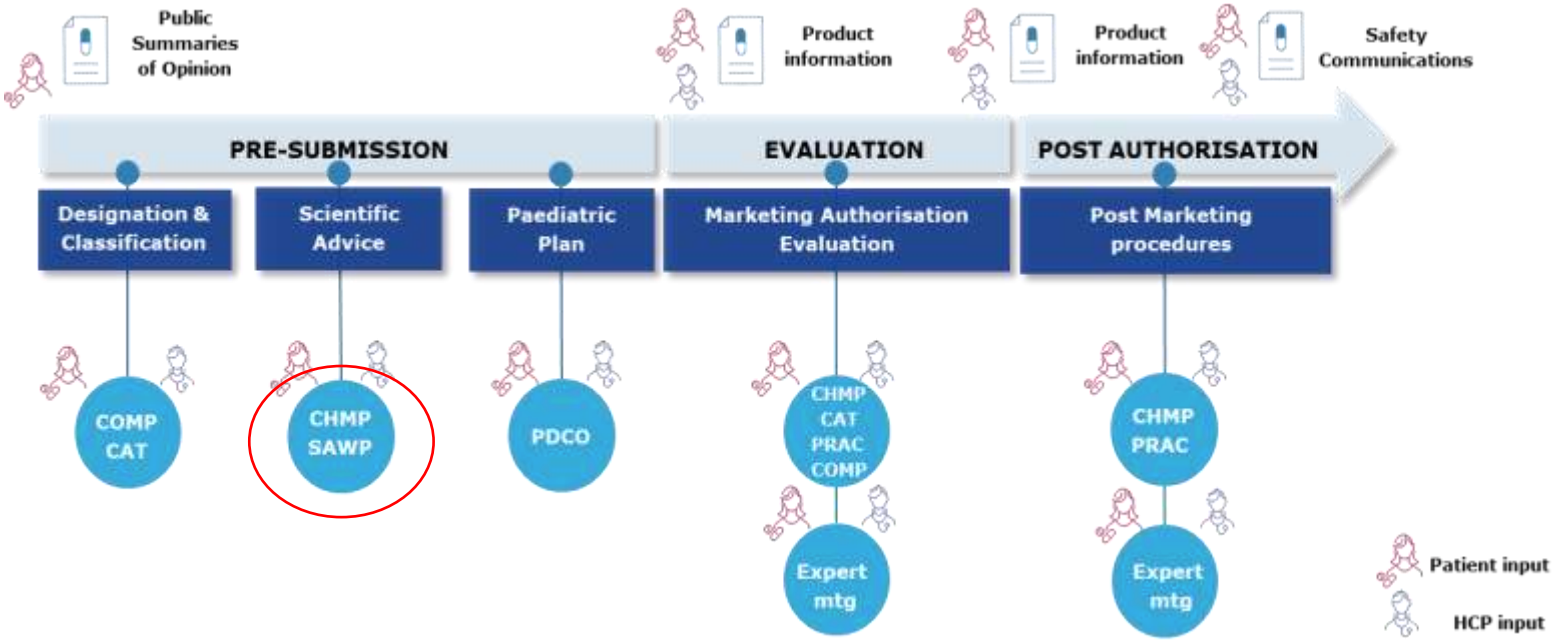
Participating in Scientific Advice procedures

What to expect and how to prepare

EURORDIS Open Academy 2025

Where are patients and HCP involved?



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
- Together with regulators, experts, including patients, and developers
- Recommendations on development for marketing authorisation

Protocol assistance is for medicines with an *orphan* designation

Scientific Advice can be provided on questions ranging from:

Quality	Manufacture of medicines
Non-clinical	animal studies, interpretation and extrapolation of results
Clinical	discussion of study population, endpoints, feasibility of trial
Regulatory	including statistics
Significant benefit	for orphan medicines (where applicable)

Clinical aspects: where you can contribute

- Selection of appropriate **end-points** (most important measures for this study):
- Defining target **population**: inclusion/exclusion **criteria**
- Choice of the right **comparator**
- Study **duration**, treatment administration, **formulation** and **dosage**
- **Clinical relevance** versus statistical significance
- Identification and assessment of **risk** potential
- **Significant benefit** (added-value) over existing therapies
- **Ethical aspects**: Informed consent

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

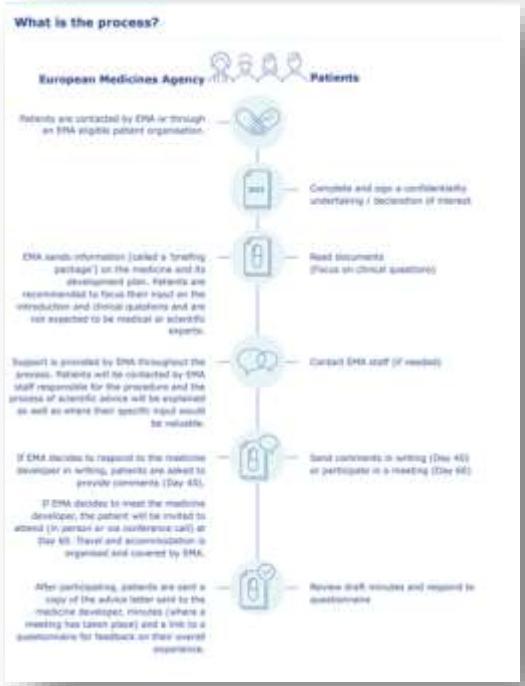
- Eligible organisations
- Previously participated
- Pool of patients – individual experts
- Check sources on internet for other diseases/conditions

Selection based on:

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

Process of involvement

- EMA: Identify expert
- Expert: Sign DOI/confidentiality
- EMA: Send package of information
- Expert: Read documents and ask questions
- Expert: Contribute to meeting or in writing



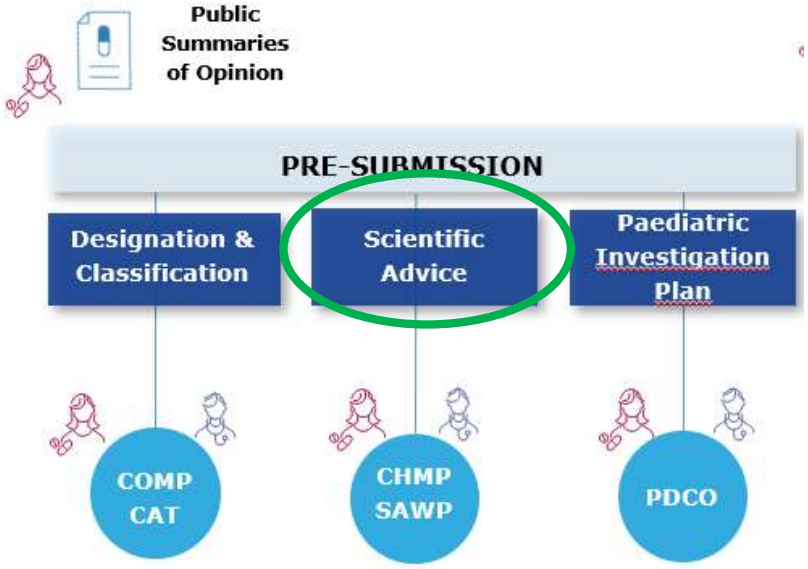
Process – competing interests/confidentiality

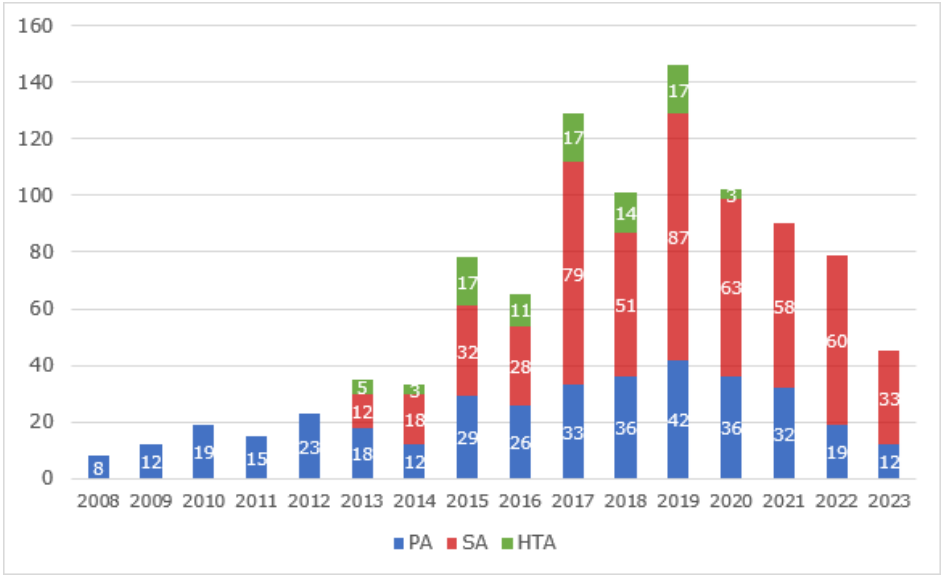
- Policy 0044 EMA competing interests
- Evaluated at the level of the individual
- Can be restrictive in some cases
- Definitions of activities not always well understood

Process – documents

- All experts receive same information
  - Lay summaries not available for patients
- All documents and meetings are in English
- Information provided on relevant sections to read
- Phone calls offered for additional support

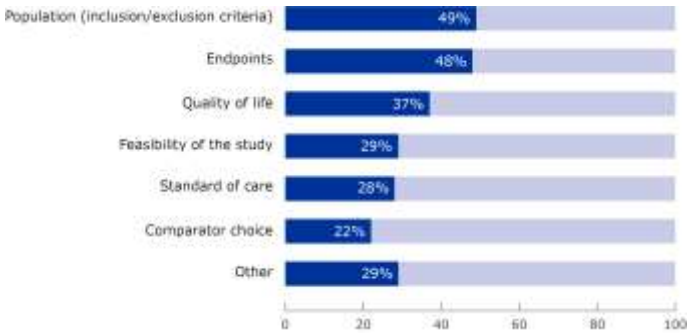
Patient Engagement in pre-submission phase: Scientific Advice





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.

Initiatives for improving contributions

Inclusion of ‘clear language’ questions for patient input

Creation of template for patient input on briefing book

Why is your contribution important?

- You know more about *living with the disease* as a patient or carer
- You know the *needs* of patients and families
- You know how your disease is *clinically managed,*
- You know where there are *unmet needs* and what is expected from innovative therapies
- You know the *feasibility* of the clinical investigations best

Take-home messages

- Process can seem daunting
- Be ready in advance of the meeting
- Focus on relevant points
- Streamline your contribution

Follow up after scientific advice

- letter of thanks; final advice letter; minutes from the meeting

Any questions?

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