

Participating in Scientific Advice procedures

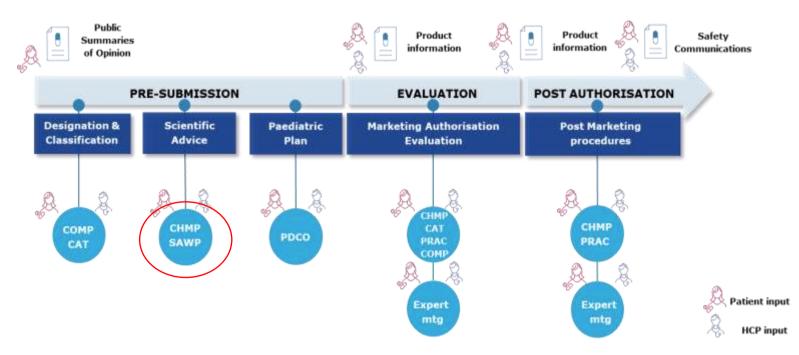
What to expect and how to prepare

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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

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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

European Medicines Agency	2222	Patients
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Process – competing interests/confidentiality



Complete and sign a confidentiality undertaking / declaration of interest

- 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

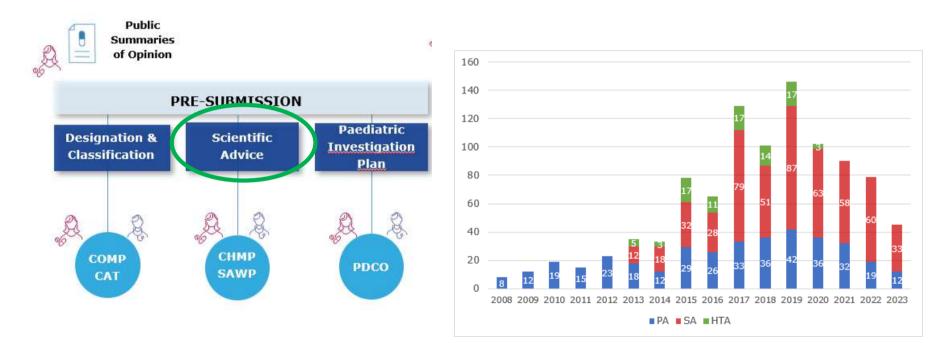


Read documents (Focus on clinical questions)

- 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

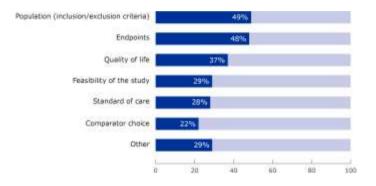


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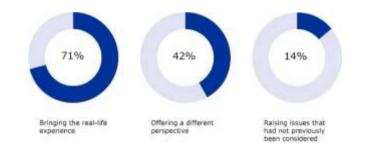
Published in Frontiers in Medicine



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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European Medicines Agency Send a question via our website

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