EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

Participating in Scientific Advice/Protocol Assistance procedures

What to expect and how to prepare

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Patient relations / Stakeholders and communication Division

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

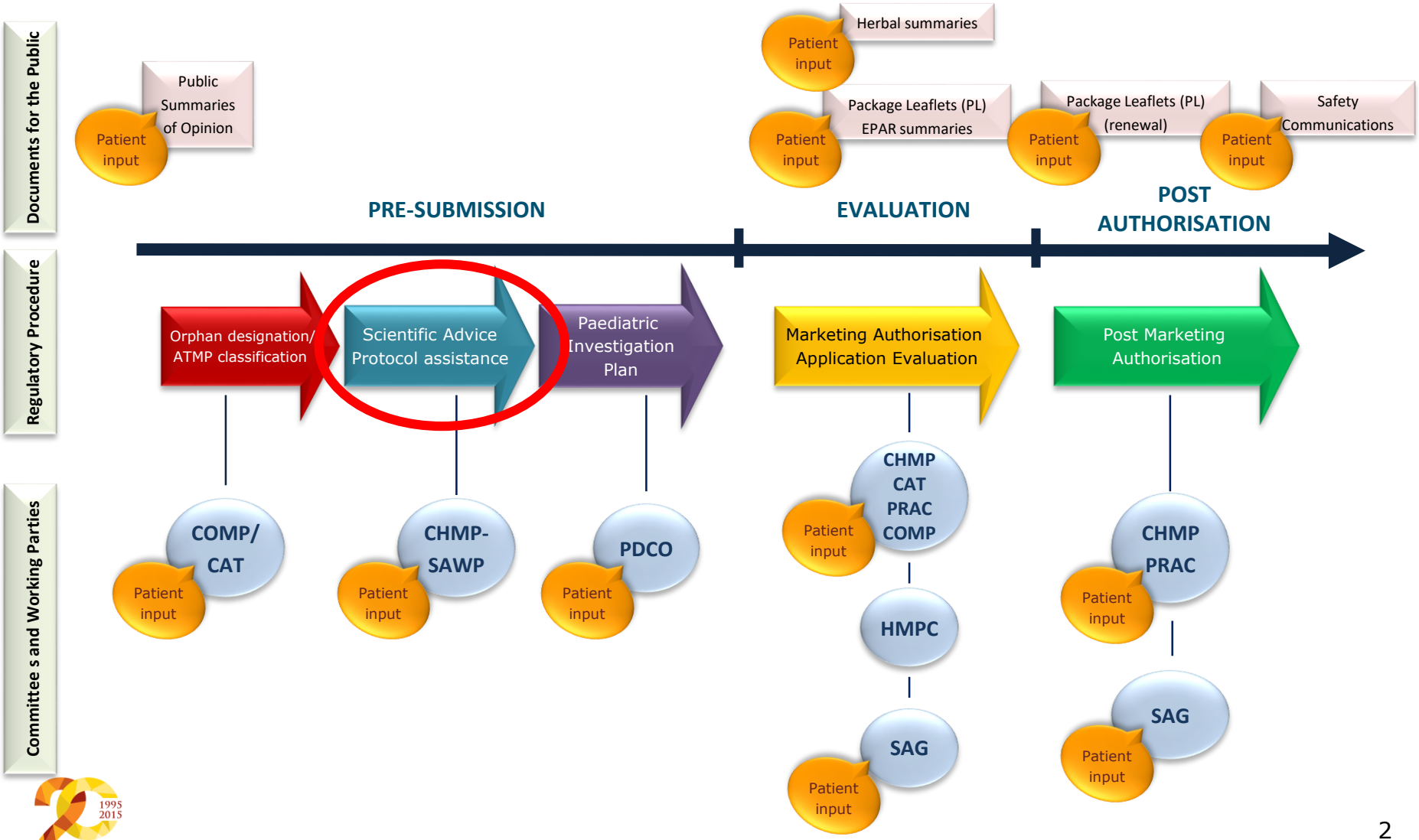
The Scientific Advice Working Party (SAWP) and the Committee for Medicinal Products for Human Use (CHMP) answer specific questions posed by the companies

Scientific advice:

- Requested by the company to the EMA (for a fee)
- Advice provided on the development of a medicine based on the documentation provided by the company
- Brings together regulators, experts, including patients, and the company
- Provide recommendations on optimising development for marketing authorisation
- Can be requested at any stage of development

Protocol assistance is scientific advice for medicines with an orphan designation

Medicines lifecycle



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)

By providing the option of scientific advice, the regulators' aim is that trials are performed to highest ethical and scientific standards required by regulators, and also efficient. Regulators are not consultants to industry

Scientific advice procedure timelines

90-day procedure

Identify procedures – identify patients – patient input into procedure

First contact

- You will be contacted either directly by the EMA (Patients and Healthcare Professionals department), **or** via your organisation
- You will receive an information sheet and video that explain scientific advice / protocol assistance and role of patients
- You will have to complete certain documents in order to participate:
    - Declaration of Interest
    - Confidentiality form
    - Nomination form

EMA – scientific advice team

- You will then be contacted by the Scientific Advice secretariat
- They will send you the documents relevant to the discussion for you to read
- The product manager will contact you to help you through the documents – you can ask any questions you may have
- Please be sure to make yourself available and speak to the product manager to be sure that you make best use of your time in preparing and contributing

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What form will your participation take?

There are two possibilities for participating:

1. Face to face meeting (Discussion Meeting)

2. Contribution in writing (Joint Report)

Face to Face (Discussion Meeting)

- There will be approximately 20-25 people in the room
  - EMA staff
  - Members of the working party
  - Representatives of the company
- The chair will introduce you and ask if you have any questions
- The company will be informed that a patient representative is present

If you cannot attend a face to face meeting with the company:

- You can still contribute in writing or join by telephone

Contribution in writing (Joint Report)

If the SAWP is responding only in writing (not meeting the company)

- You can still contribute your perspective by commenting on the proposed advice in writing
- Timelines are shorter

Where can you contribute; clinical aspects:

- Selection of appropriate end-points:
- 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

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

Follow up after scientific advice

Once the scientific advice has been provided, you will receive a letter of thanks and a copy of the final advice letter and minutes from the meeting

Take-home messages

- Process can be seem daunting
- Be ready and prepare yourself in advance of the meeting
- Focus on relevant points of the clinical investigation
- Streamline what your contribution can be
- Patient input is another piece of the puzzle
- Patient input makes a difference

Contact

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