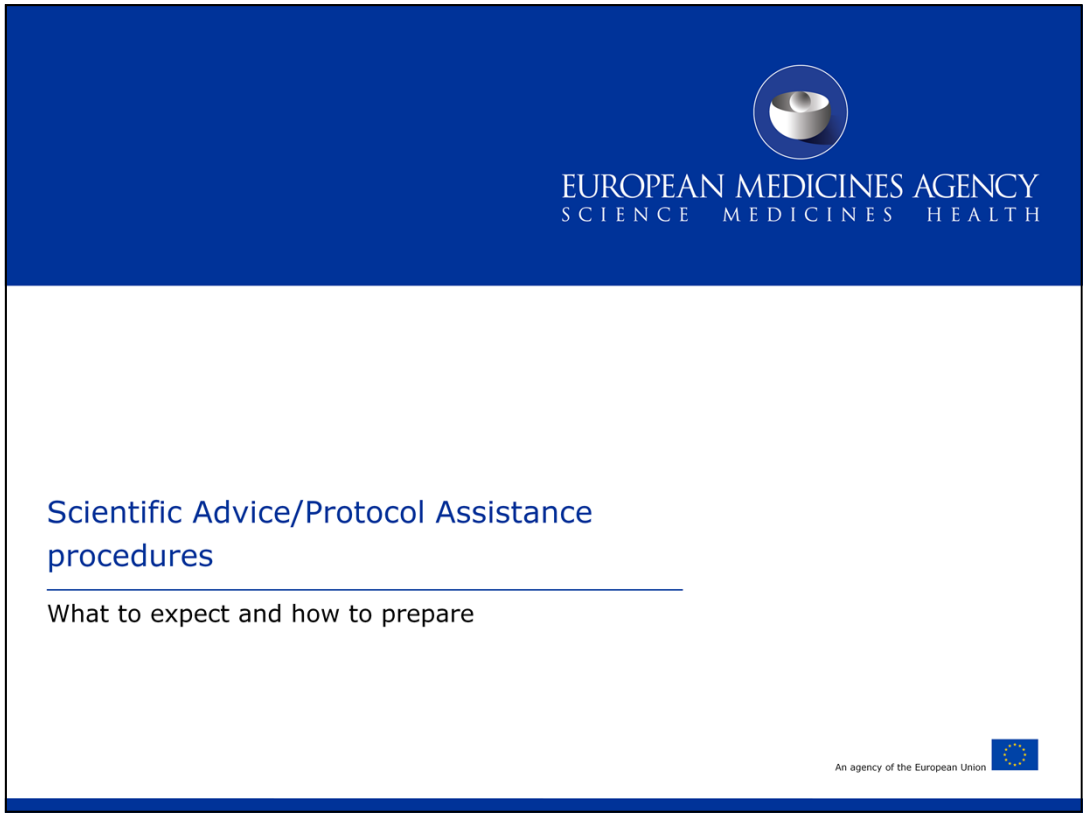
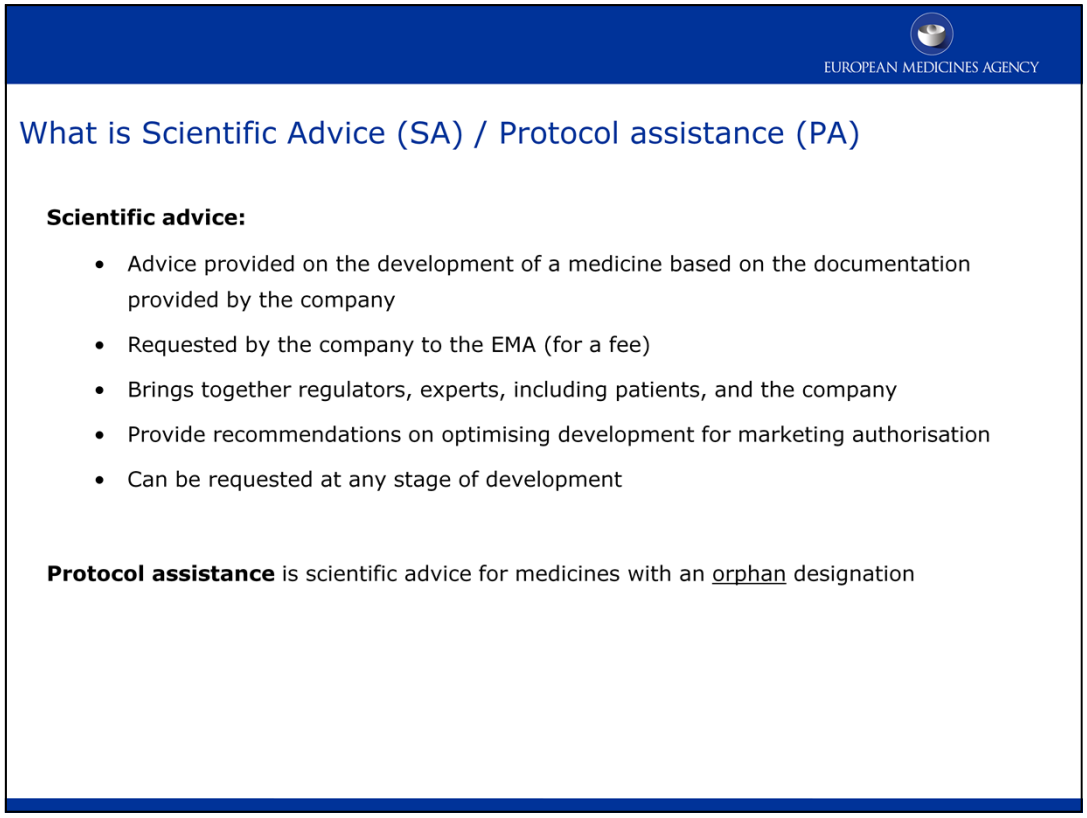


In this video we will describe the process of scientific advice as well as how patients are involved and how they can best prepare for this activity



But first, what is scientific advice?

Scientific advice is the advice provided by the EMA upon the request of companies on the development of a medicine.

The companies provide information in a question and answer format and a fee is charged for this activity.

Protocol assistance is scientific advice for medicines that have received an orphan designation (that are for the treatment of rare diseases)

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Scientific Advice Working Party (SAWP)

- EU members nominated from national agencies and academia
- Multidisciplinary: non clinical safety, pharmacokinetics, methodology and statistics, diverse therapeutic areas
- Cross-committee: CHMP, COMP, PDCO, CAT
- Patient involvement
- Monthly face-to-face meeting at the EMA
- Letter Peer reviewed and adopted by CHMP
- Significant benefit answer adopted by COMP
- Advice is not binding however statistics show that companies that follow scientific advice are more likely to have a positive authorisation

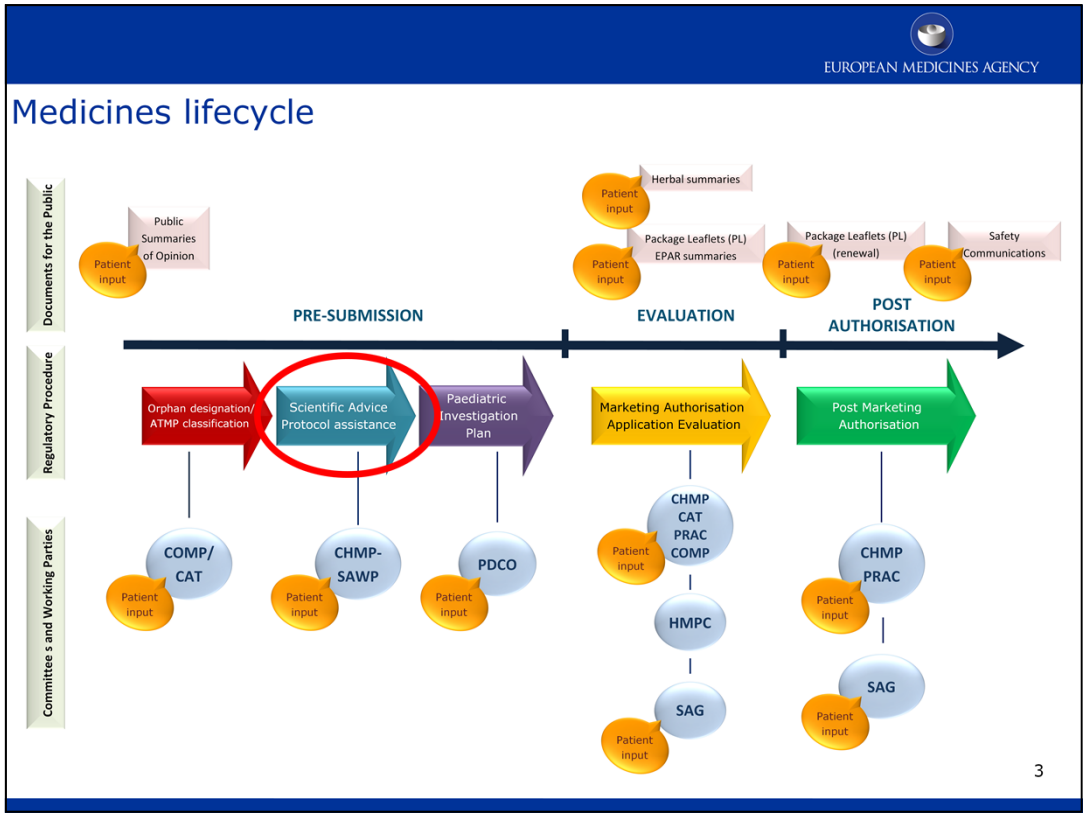
2

Scientific advice and Protocol Assistance are coordinated by the Scientific Advice Working Party

The working party is made up of EU members who are either nominated from national agencies or come from academia. These members are selected for their experience in the different areas of medicines development.

In addition members from other EMA committees are also included who are responsible for particular aspects of the advice provided.

Patients with experience of a particular disease can also be invited.



If you have watched the other videos, you will be familiar with this figure,

This diagram shows an overview of the medicines regulatory pathway and you can see that it is split into Pre-Submission, Evaluation and Post-Authorisation.

Here we see where in the process scientific advice occurs

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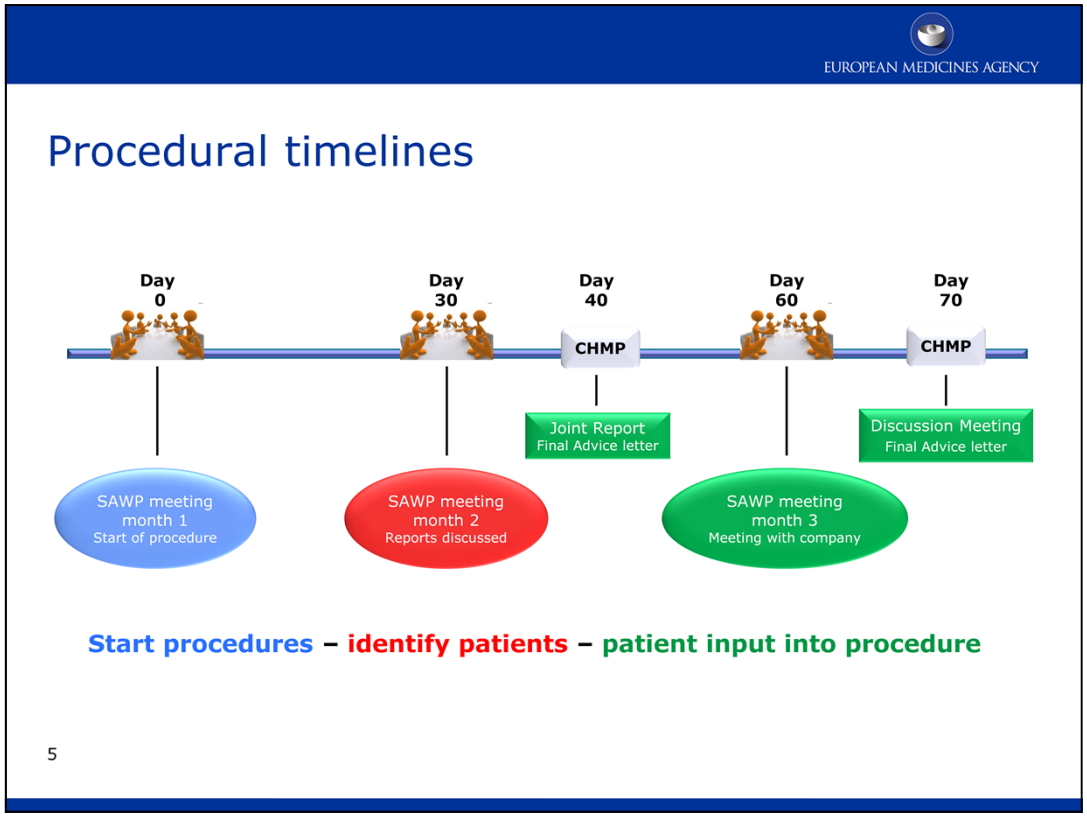
Scientific Advice can be provided on :

- **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)

4

Scientific Advice covers all aspects of development of a medicine from quality of the manufacturing process to non-clinical questions, clinical aspects and regulatory and significant benefit for orphan designated medicines

By providing the option of scientific advice, the regulators' aim is for trials to be performed efficiently and to the highest ethical and scientific standards.



The scientific advice procedure takes 60 days

On Day 0 the working party meets and allocates coordinators for the questions asked by the companies.

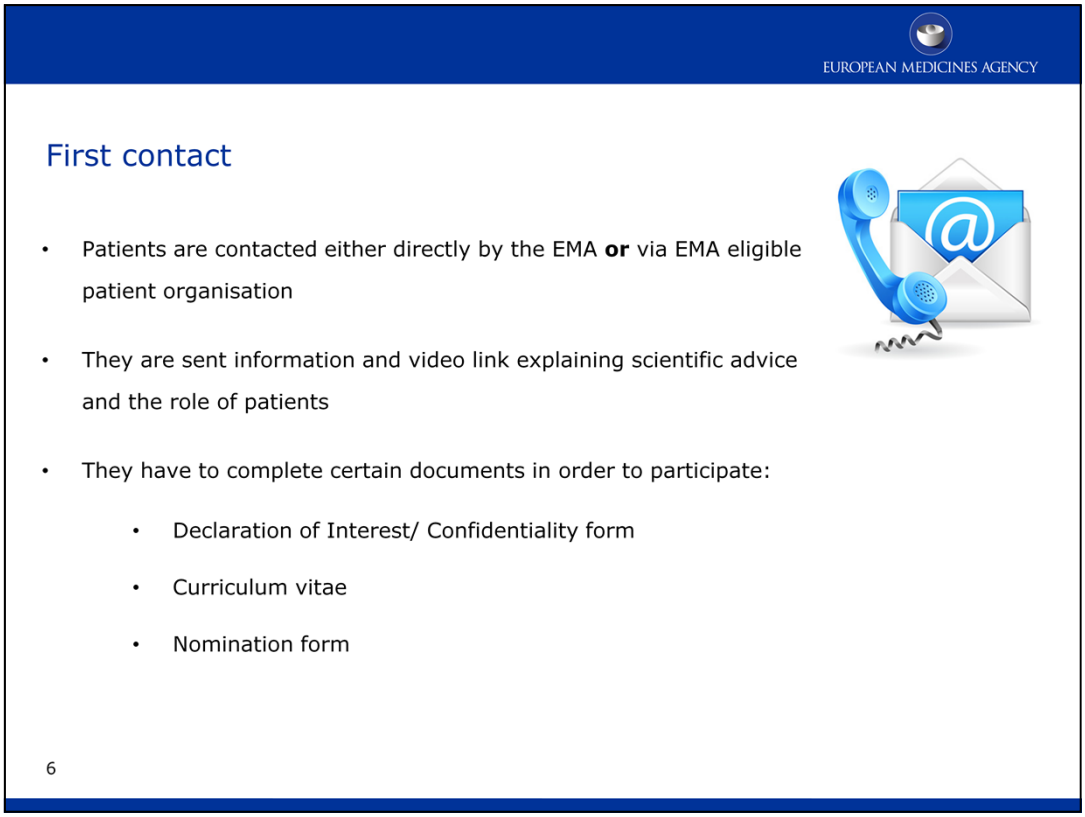
At the same time the dossiers that would best benefit from the input of patients is communicated to the EMA team responsible for patients.

On Day 30 the decision is made by the working party on whether they need to meet the company or if they will respond in writing.

As you see the timelines for responses in writing are shorter (day 40).

The working party meets with the company on day 60

Patients invited to contribute in both cases.



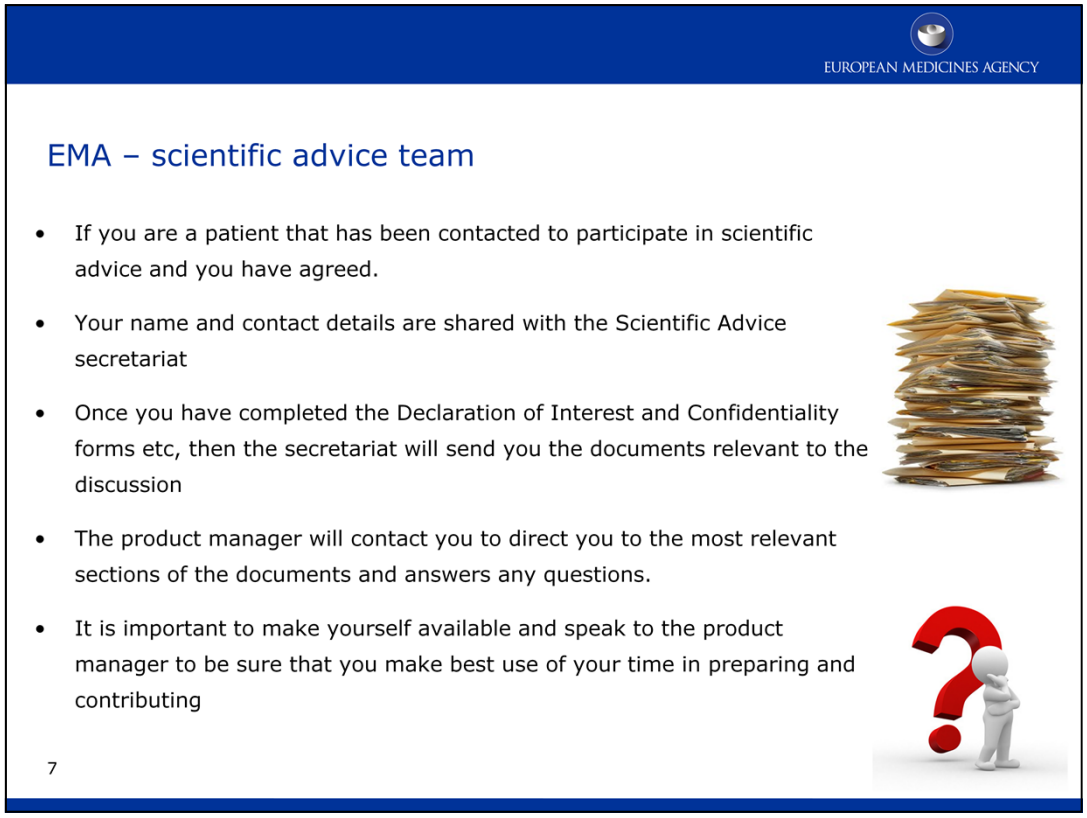
Patients are contacted to participate in scientific advice either directly by the EMA **or** via an EMA eligible patient organisation

Patients are sent information and video explaining scientific advice and their role

Like all experts, they have to complete certain documents in order to participate:

- Declaration of Interest/ Confidentiality form
- Curriculum vitae
- Nomination form

Please see the EMABasic video on how to complete the Declarations of Interest : a practical guide



- If you are a patient that has been contacted to participate in scientific advice and you have agreed.
- Your name and contact details are shared with the Scientific Advice secretariat
- Once you have completed the Declaration of Interest and Confidentiality forms etc, then the secretariat will send you the documents relevant to the discussion
- The product manager will contact you to direct you to the most relevant sections of the documents and answers any questions.
- It is important 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

EUROPEAN MEDICINES AGENCY

EMA – scientific advice team

- Once a patient has been identified, their name is shared with the Scientific Advice secretariat
- Once the Declaration of Interest and Confidentiali[illegible] ed, then the secretariat sends the documents rele[illegible]
- The product manager co[illegible] [illegible]p focus the patient to the most relevant se[illegible] [illegible]s and answers any questions.
- For patie[illegible] [illegible]tant to make yourself available and speak to the product ma[illegible]ger to be sure that you make best use of your time in preparing and contributing

CONFIDENTIAL

8

- Treat all documents with the utmost confidentiality – this means that you can not share or discuss the contents with anyone

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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)

9

The scientific advice working party will decide on Day 30 of the procedure (as seen earlier) whether they want to meet the company or respond in writing.

You may be invited to participate in either outcome.

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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
  - Patient
- The chair will introduce you and ask if they 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

10

In the case of a discussion meeting where the company comes to the EMA,

there will be approximately 20-25 people in the room including EMA staff, members of the working party and representatives of the company.

You will be introduced to the other members of the working party and EMA staff. You can ask any questions you like.

The company is invited into the room and informed that a patient is present and the discussion begins. If you can not attend in person, you can either participate by telephone or send comments in writing.

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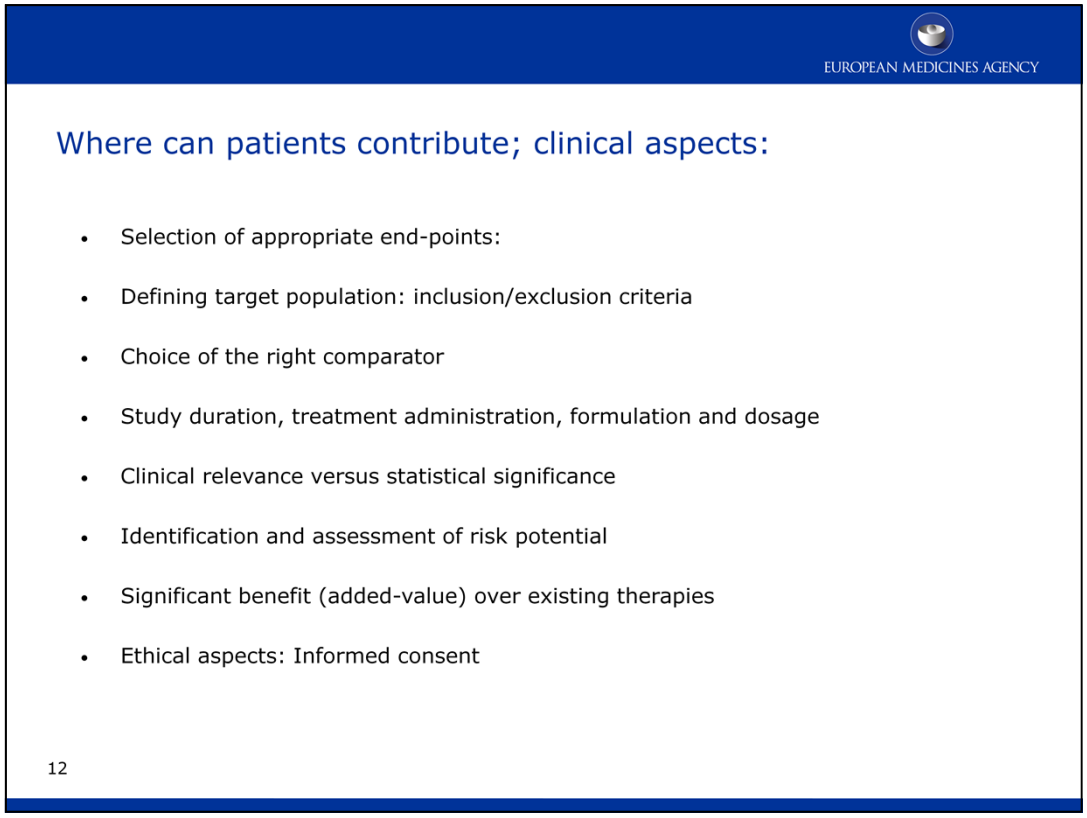
Contribution in writing (Joint Report)

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

If the working party is responding only in writing (that is, not meeting the company)

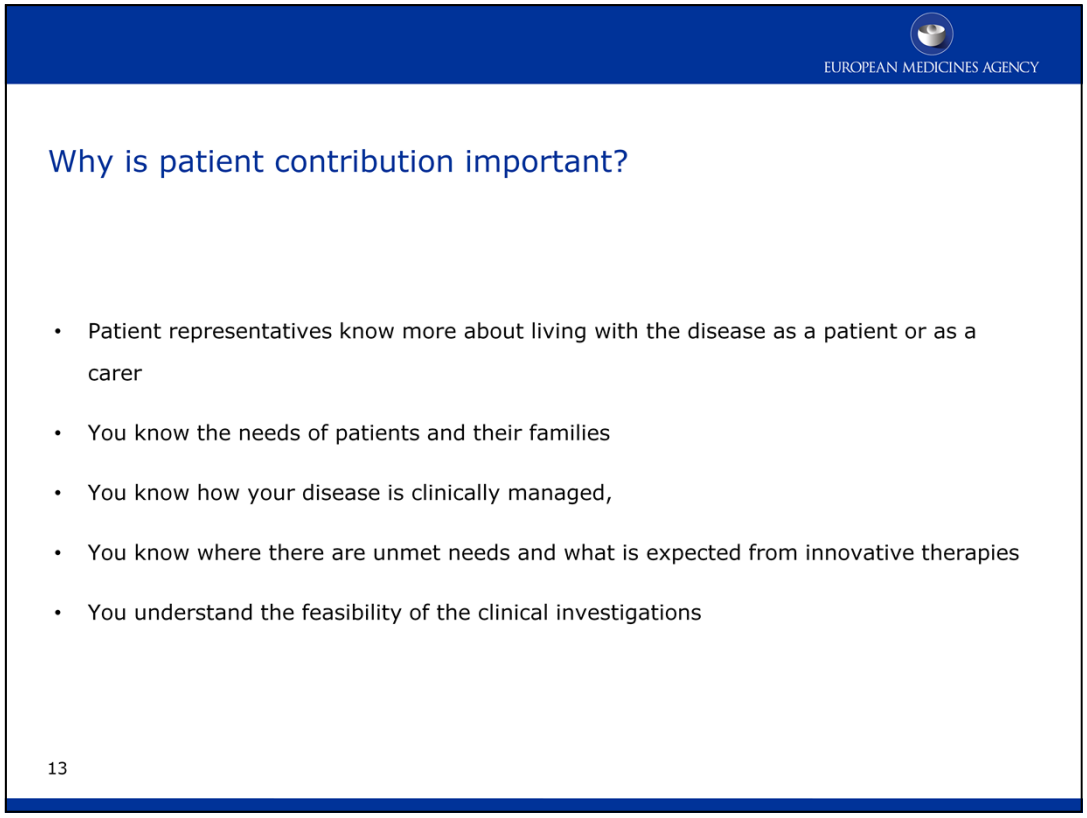
- You can still contribute your perspective by commenting on the proposed advice in writing
- Please note that the timelines are shorter



Some of the areas where you can contribute to the scientific advice requested by a company on their development plans include:

- The Selection of appropriate end-points:
- Defining the target population
- Choosing the right comparator
- The duration of the study, treatment administration, formulation and dose of the medicine
- The Clinical relevance versus the statistical significance
- Identification and assessment of risk potential

- Significant benefit (added-value) over existing therapies in the case of orphan medicines
- You can also comment on Informed consent



Why do we need the input of patients?

Patients know more about living with the disease as a patient or carer

- You know the needs of patients and their families
- You know how your disease is clinically managed,
- You know where there are unmet needs and what is expected from innovative therapies
- You understand the feasibility of the clinical investigations

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Follow up after scientific advice

Once the scientific advice has been provided, a letter of thanks and a copy of the final advice letter and minutes from the meeting is sent to the patient

Take-home messages

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

14

Once the meeting is finished, the working party will reconvene to discuss the outcome and will prepare a final advice letter – this letter needs to be signed off by the committee for human medicinal products (or CHMP).

You will be sent a copy of this letter, as well as minutes from the meeting and a letter of thanks for your participation.

It is important to remember that although the process can seem daunting, your input is highly valued – It is important to prepare for the meeting, to focus on the relevant points of the discussion and to streamline your contribution.

Patient input is an equal part of the whole picture and your input does make a difference.

If you would like to know more about scientific advice, go to the EMA website at www.ema.europa.eu

If you would like to register to be involved in EMA activities as a patient expert, please go to the Getting Involved section of the patients' pages on the EMA website or click on the link in the pdf version of this talk.