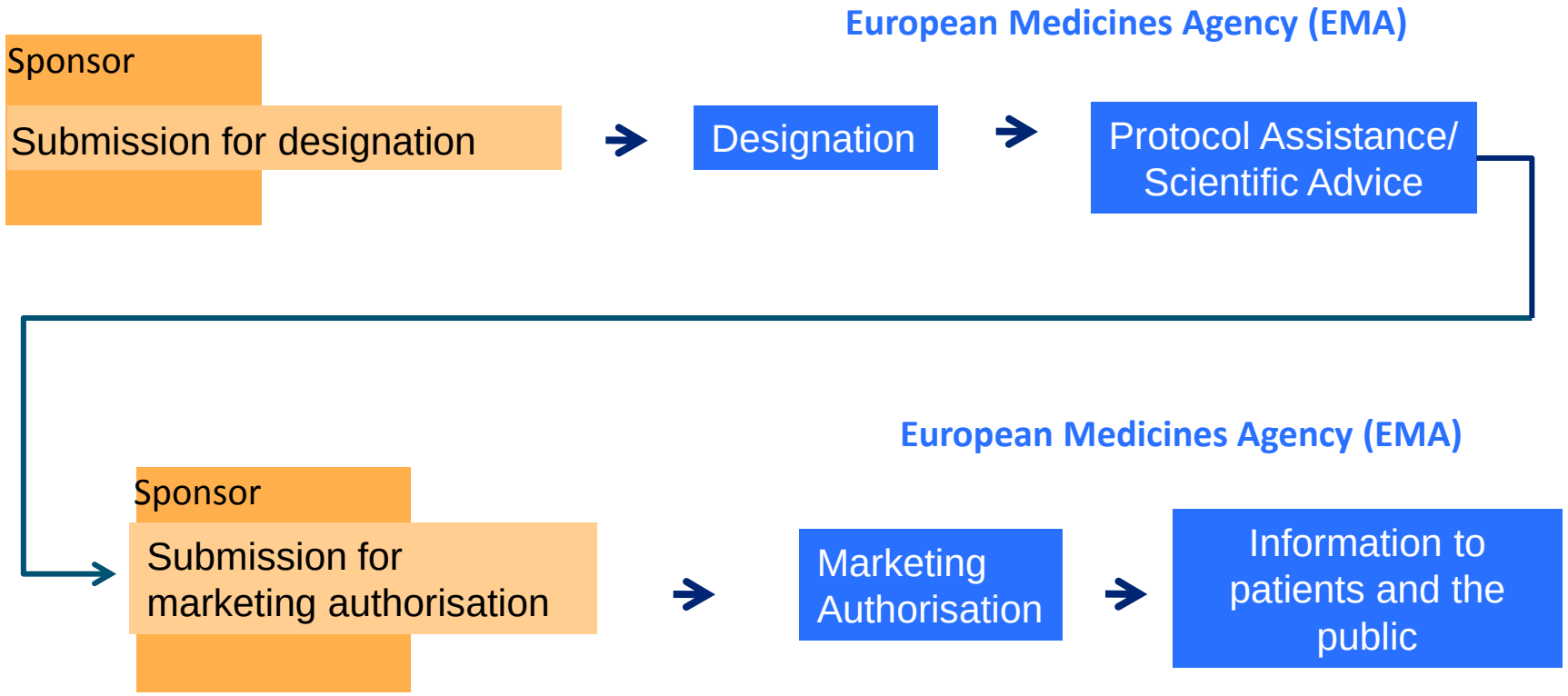
EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

Scientific Advice/Protocol Assistance

What to expect and how to prepare

Presented by Maria Mavris on 5 June 2015
Patient Relations

Regulatory pathway for an orphan product: the context



Timeline of scientific advice procedures

Identify procedures – identify patients – patient input into procedure

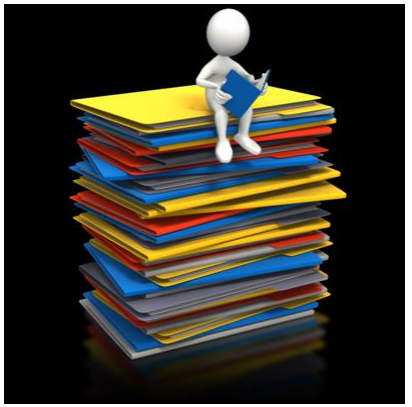
What to expect..

- You will be contacted either directly by the EMA, EURORDIS or via your organisation
- You will receive an information sheet and video that explain scientific advice and role of patients
- You will have to complete certain documents in order to participate:
    - Declaration of Interest
    - Confidentiality form
    - Nomination form

What to expect..

- You will then be contacted by the Scientific Advice Working Party (SAWP) 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

What to expect..

There are two options for participating:

- Face to face meeting

- Contribution in writing

- Once the scientific advice has been provided, you will receive a letter of thanks and a copy of the final advice letter.

What to expect..

If you attend a face to face 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 (before the company arrives) and ask you if you have any questions
- When the company arrives they will be informed that a patient representative is present
- Feel free to contribute when you feel appropriate

What to expect..

If you can not attend a face to face meeting with the company:

- You can still contribute in writing or join by telephone

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

- You can still contribute your perspective
- Timelines are shorter

How can you be involved?

- You know more about *living with the disease* as a patient or carer than the medical or scientific experts
- You know the *needs* of your patients and families best
- You know how your disease is *clinically managed* and who are the "true" clinical experts and where they work.
- You know about the *lack of treatment* and what can be expected from innovative therapies
- You know the *feasibility* of the clinical investigations best

Types of questions

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)

Take-home messages

- Process can be seem daunting
- Patient input is another piece of the puzzle
- Patient input is impactful