EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

Scientific advice/protocol assistance and patients

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Training session for patients/consumers involved in EMA activities

Presentation Outline

- What is scientific advice
- When/Why will patients be involved
- Numbers so far
- How can patients contribute to benefit/risk discussion
- Case examples

What is scientific advice?

Voluntary procedure:

- Applicants ask questions through a scientific advice/protocol assistance procedure
- Can be sought at any stage of drug development and on any area of development (quality, non-clinical or clinical)
- Fee-based

Provided by the Scientific Advice Working Party (SAWP):

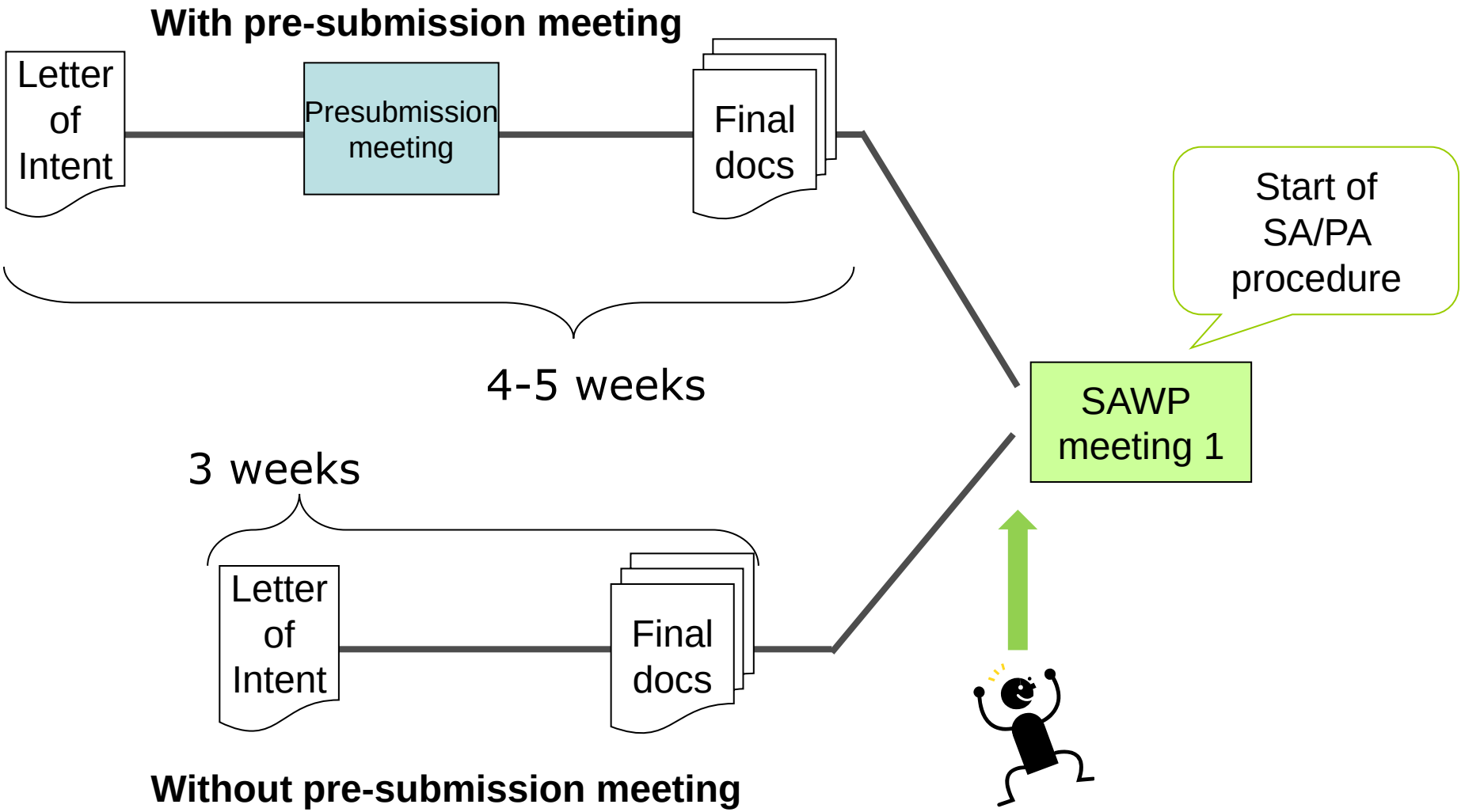
- 30 experts from national authorities, university departments and hospitals, chosen by required expertise.

Supported by the EMA Secretariat

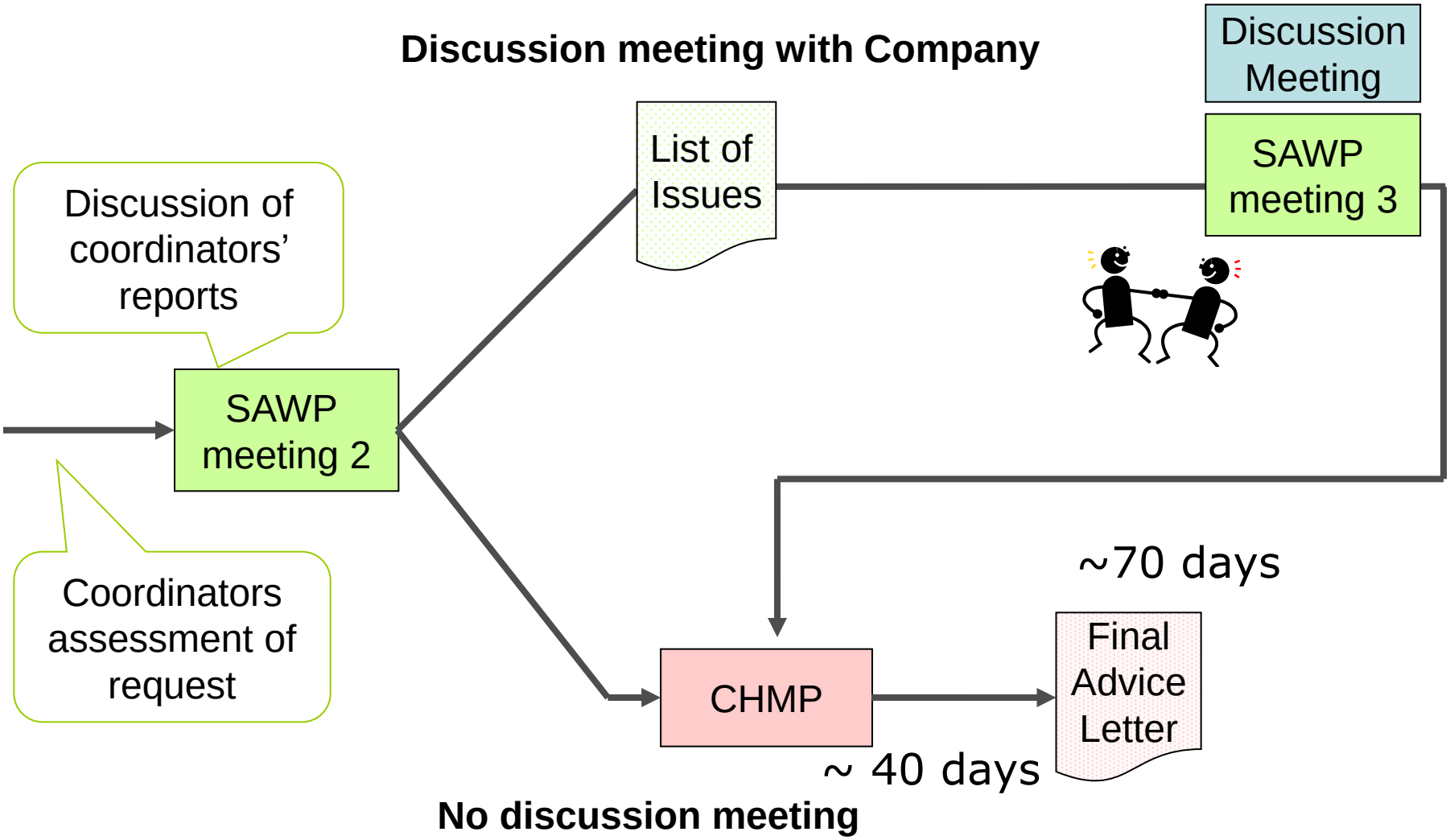
How is it given?

- Applicant submits the questions and supportive documentation
- 2 Coordinators from the SAWP write the report
- In 50% of the cases, in particular when the regulatory experts do not agree with the Applicant's proposal, **a face-to-face meeting** with the company is organised
- Written responses are adopted by the CHMP and send to the Applicant: scientific advice letter
- Short procedure: 40 days or 70 days when a face-to-face meeting takes place.

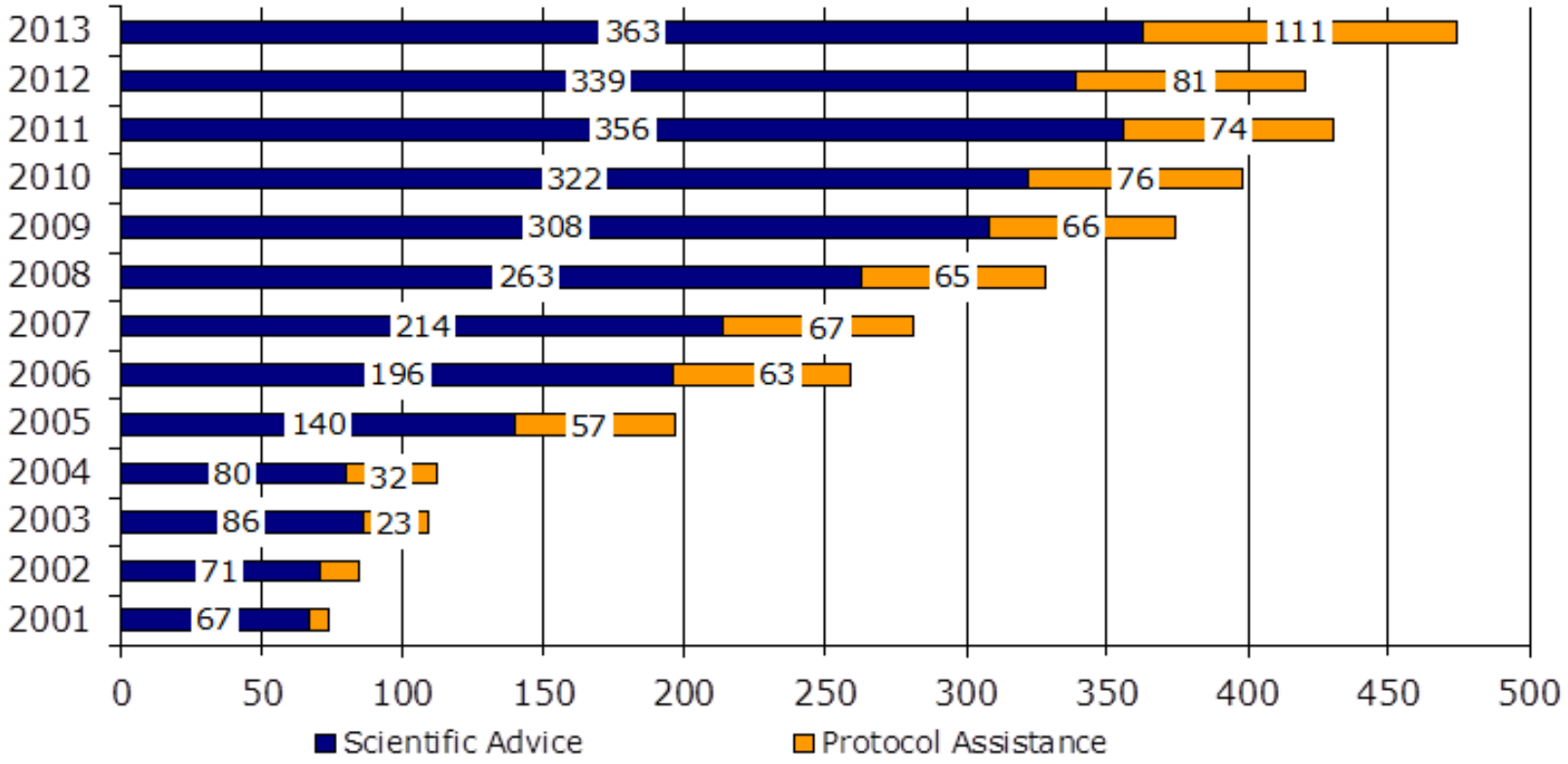
Procedure workflow



Procedure workflow



Scientific Advice main activity so far: product related scientific advice and protocol assistance for orphan drugs



Types of Scientific Advice and related procedures

- Scientific Advice (SA)
- Protocol Assistance (PA)
- Biomarker Qualification
- Parallel SA/health technology assessment (HTA)
- Adaptive Licensing

Numbers for patient representatives

Protocol assistance	2008	2009	2010	2011	2012	2013	2014 – to date
Total no of PA procedures in total for the year	56	77	68	74	60	111	
Number of PRs involved	8	13	18	16	19	16	11

Biomarkers	2013	2014- to date
Total no of Biomarkers procedures in total for the year	15	
Number of PRs involved	4	2

HTA: 2 procedures in 2014

When/Why will patients be involved

Scientific advice / Protocol assistance

- Try to include patients representatives, where appropriate
  - Some procedures can be very technical (i.e. on quality and non-clinical aspects) or follow-up advice (i.e. confirming previous agreement)
- Particularly valuable in case of face-to-face meeting with the Applicant.
- We value your input!

Qualification of biomarkers: genomics, imaging, **scales, patient reported outcomes (PRO)**

Adaptive Licensing

Support the definition of pathway of product development and (potential) earlier access to medicines through early dialogue involving all stakeholders (regulators, HTAs, payers, patients…)

Criteria for candidate selection

1. An iterative development plan (start in a well-defined subpopulation and expand, or have a Conditional Marketing Authorisation, maybe surrogate endpoints and confirm)
2. Real World Data (safety and efficacy) can be acquired to supplement Clinical Trials
3. Input of all stakeholders, particularly HTAs, is fundamental

Unmet medical need is an important feature that allows full use of regulatory tools

How can patients contribute to benefit/risk discussion

Case by case but in general:

- To add your views on the issues being discussed e.g.
  - Feasibility of the study proposed
  - Relevant patient population
  - Comparator or not
  - Duration of study
  - Relevant patient outcomes
  - Safety concerns

- Add additional comments on the development.

- In writing and/or in person/TC if a discussion meeting takes place.

Real life examples/personal experience where patients involved

Example 1: Product for cystic fibrosis

A discussion of the products ability to improve fat and protein absorption.

Patient rep confirmed that fat absorption is the **worst symptom of the disease**.
Patient rep asked whether product lacking a protease component would be a risk for physically active CF patients.

Example 2: Enzyme replacement therapy

Comments from patient rep on aspects of a clinical trial in a lysosomal storage disorder which would be beneficial for the patients:

- Reduced infusion times
- Fewer adverse events
- Less likely to have off-target effects
- Less likely to create antibody reactions that diminish the drug efficacy
- Reduced pharmacy time to reconstitute drug
- More flexibility for clinicians to change drug frequency

Example 3 treatment for rare paediatric cancer

The Applicant proposed a single arm pivotal study which the SAWP questioned:

Patient /physician confirmed that no patients would be enrol in a placebo comparative study. Since there are several competing studies the parent would bring their child to another investigator/study.

The SAWP agreed to the single arm study.

Further information

Home EMA> Regulatory > Human medicines > Scientific advice and protocol assistance

We very much welcome patients additional involvement and look forward to seeing more patient representatives in scientific advice.

Questions?

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