EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

Scientific advice / protocol assistance and patient representatives

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Training session for patients/consumers involved in EMA activities

Plan

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

What is scientific advice

Voluntary procedure:

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

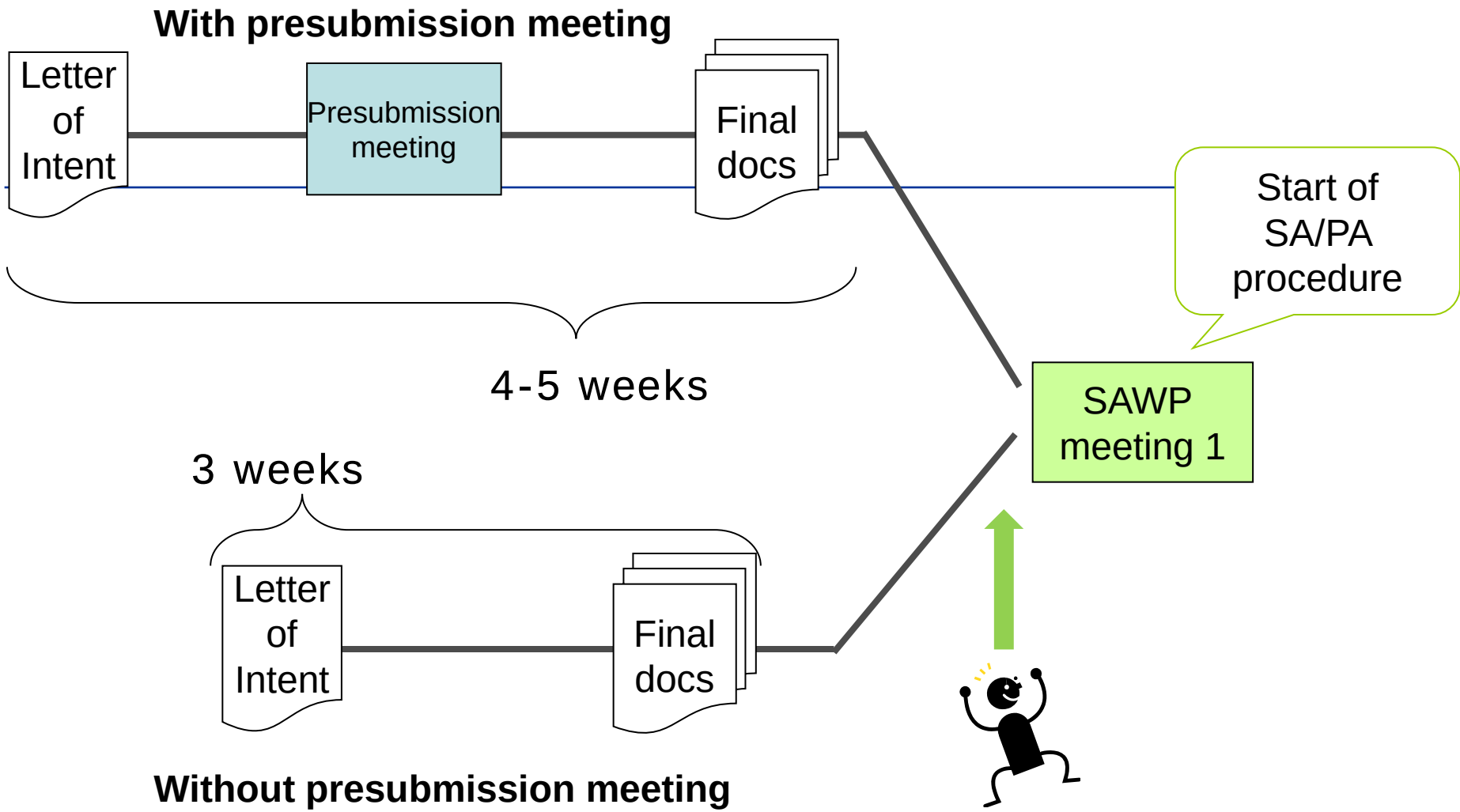
Given by the Scientific Advice Working Party (SAWP):
30 experts from national authorities and university departments and hospitals chosen by required expertise.

Supported by the EMA Secretariat

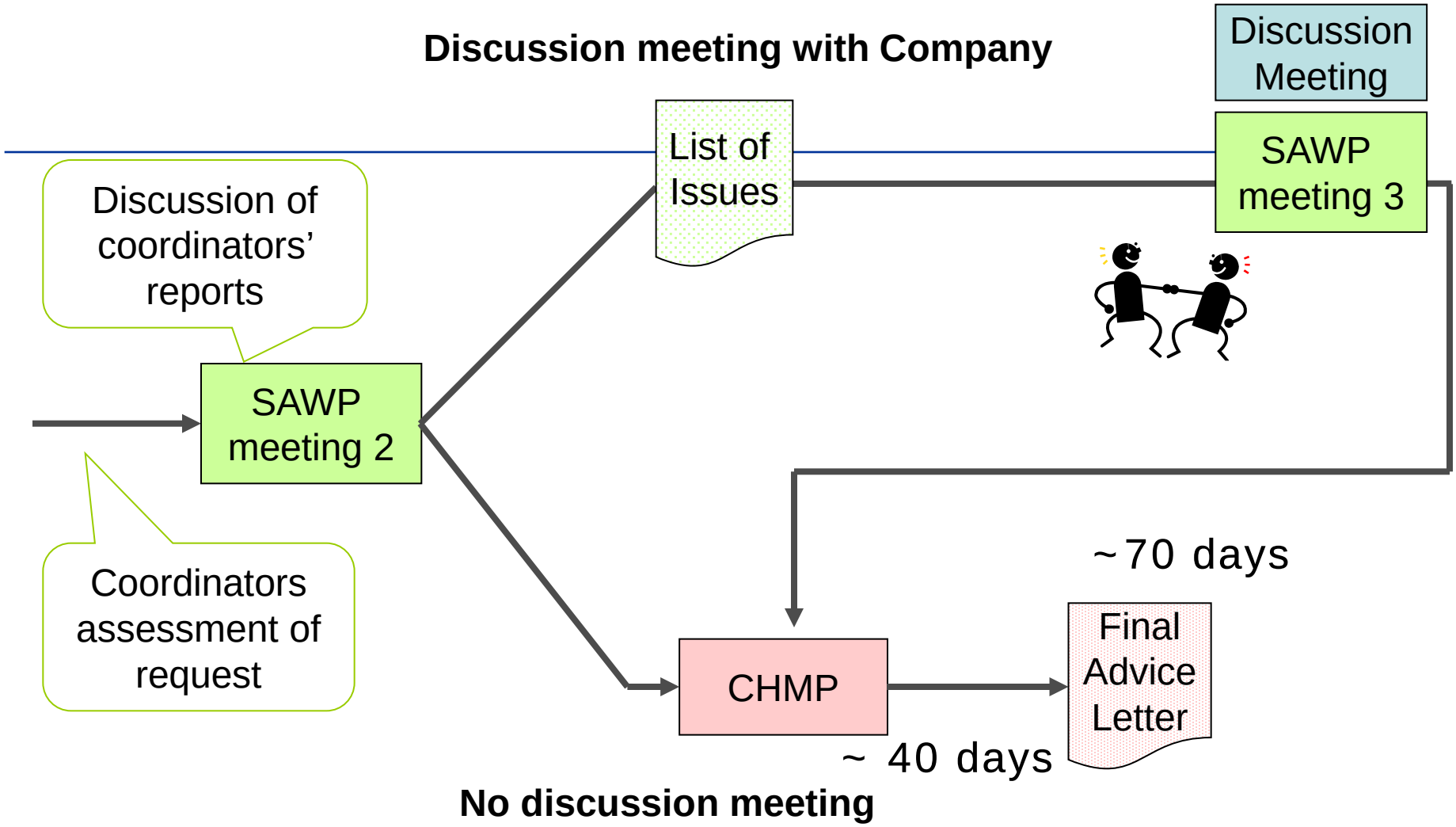
How is it given

- Applicant submits the questions and supportive documentation
- 2 Coordinators from the SAWP writes 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.

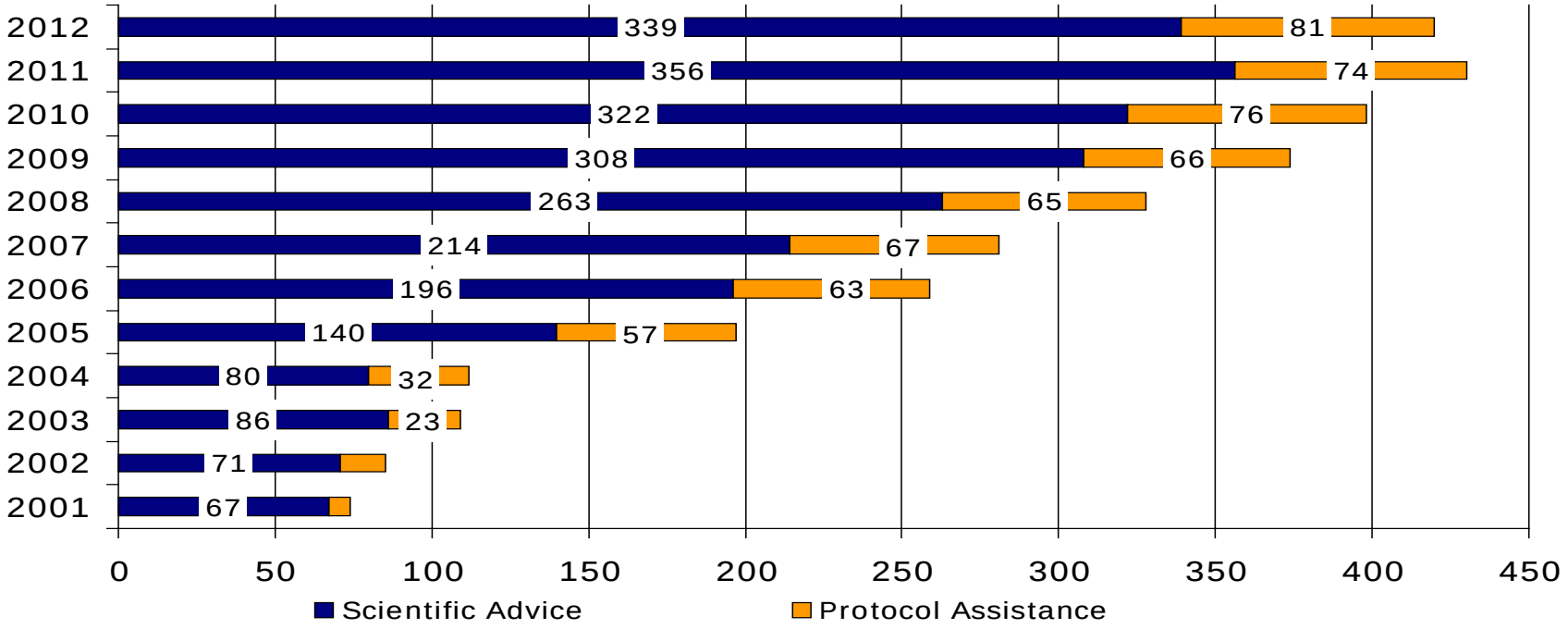
Procedure workflow



Procedure workflow



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



Numbers so far for patient reps

Protocol assistance	2008	2009	2010	2011	2012	2013
Total no of PA sent to Eurordis	56	77	68	74	60*	24
Number of PRs identified	-	-	-	20	22	18
Number of PRs involved	8	13	18	16	19	16

Scientific advice	2013
	30 procedures identified, 23 identified patient reps (7 will participate in meeting next year) and in total 12 patient reps participated during 2013.

When/Why will patients be involved

Scientific advice / Protocol assistance

- We attempt to include patients representatives for all procedures.
- Not all procedures are regarded as candidate for patient reps to participate e.g. the advice can be very technical on quality and non-clin only, follow-up advices only confirming previous agreement etc.
- Patient rep will be involved from start of the procedure and in particular valuable in case of a face-to-face meeting with the Applicant.

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

1 procedure so far including 2 patient reps.

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 rep/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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