EURORDIS
Rare Diseases Europe

Scientific Advice / Protocol Assistance:

experience and impact of patient involvement

Outline

- Elaboration of survey with EMA
- Selection of patients' representatives for survey
- Presentation of results
  - Benefit Risk
- Conclusions/suggestions
- Extra slides (additional information on support aspects)

Survey

- Survey designed between EURORDIS and European Medicines Agency
- 15 questions in total
  - Patient information, preparation and support
  - Contributions to Benefit/Risk discussions

Survey questions – participation /support

1. Had you heard about protocol assistance prior to being contacted to be involved?

- ○ Yes
- ○ No

2. Did you receive enough information, regarding the scientific process and logistics, at the time you were contacted to participate?

- ○ Yes
- ○ No

3. If you answered No to question 2, please explain what additional information you would have liked to receive.

4. Prior to your participation in protocol assistance, did you receive enough support from:

	Yes	No
EURORDIS?	○	○
the European Medicines Agency?	○	○
Your association?	○	○

5. What additional support would you have liked?

Survey questions

6. How would you rate your experience with protocol assistance?

☐ Interesting ☐ Beneficial ☐ Helpful ☐ Waste of time ☐ Frustrating

7. During the meeting, did you have an opportunity to make a contribution?

◯ Yes

◯ No

8. Did you feel your presence at the meeting (including teleconference or written contribution) was beneficial to:

	Yes	No
the process?	◯	◯
to you?	◯	◯
to the patients you were representing?	◯	◯

Survey questions

9. Were the benefits and risks of the proposed medicine discussed?

- ○ Yes
- ○ No

10. Did you participate in these discussions?

- ○ Yes
- ○ No

Other (please specify)

11. If you answered NO to the question 10, please explain why

12. Was the discussion on benefits an risks constructive?

- ○ Yes
- ○ No

Survey questions

13. Can you describe (within limits of confidentiality) the contribution to the discussions on benefit risk that you made?

14. Do you feel that you opinion as a patient was (or should have been) an important element of the benefit/risk discussions?

- ○ Yes
- ○ No

Other (please specify)

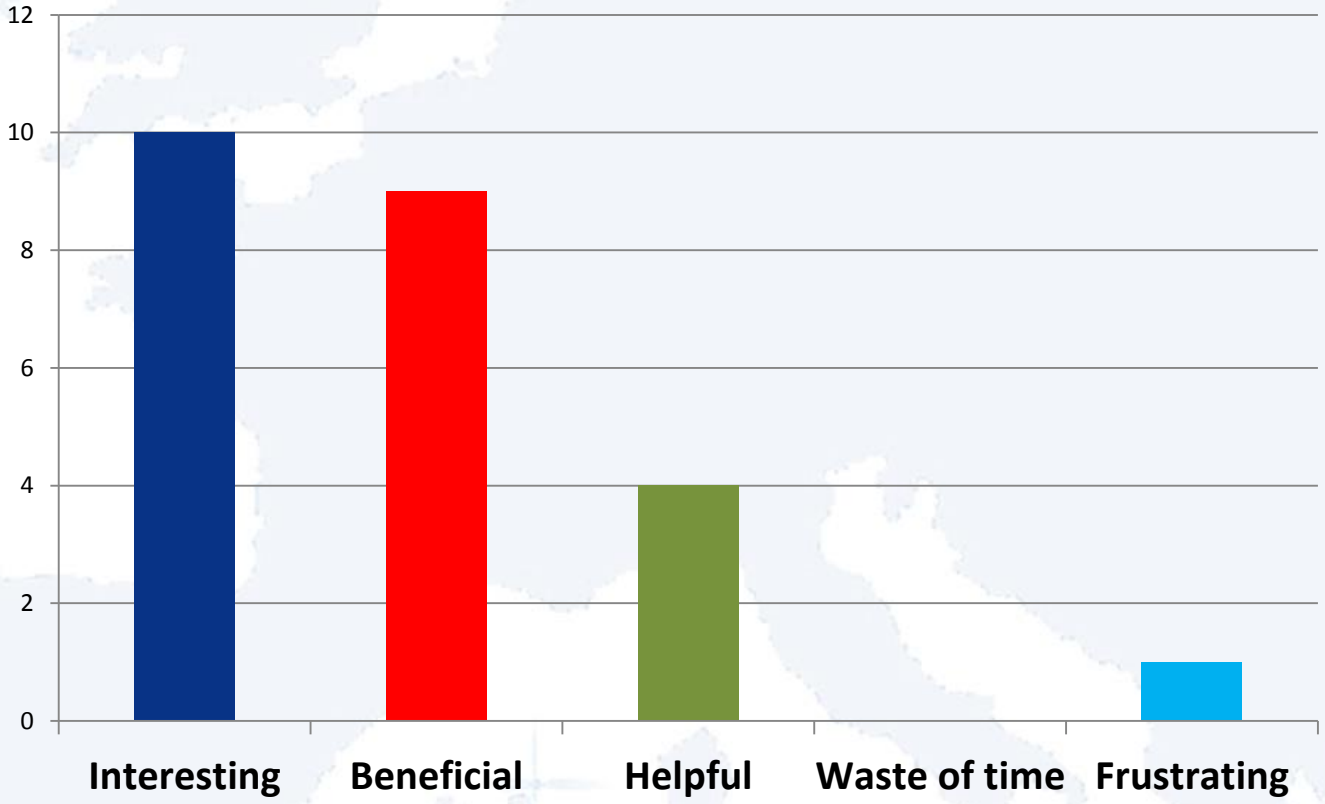
15. Do you have any suggestions on how the discussions on benefits and risks could be improved to better include the patient viewpoint?

Survey participants

- 18 patients' representatives selected - 14 responses
- Some patients had participated in PA more than once.
- Diseases represented (15):
  - alpha-1 antitrypsin deficiency, Behçets disease, cushing's syndrome, cystic fibrosis, cystinosis, epidermolysis bullosa, Leber's congenital amaurosis, mucopolysaccharoidosis, pulmonary arterial hypertension, retinitis pigmentosa, spinal cord injury, spinal muscular atrophy, thalassaemia, tuberous sclerosis complex, Wegener's granulomatosis

Responses to survey – participation/support

Q6. How would you rate your experience with protocol assistance?



Responses to survey – benefit/risk input

Q9. Were the benefits and risks of the proposed medicine discussed?

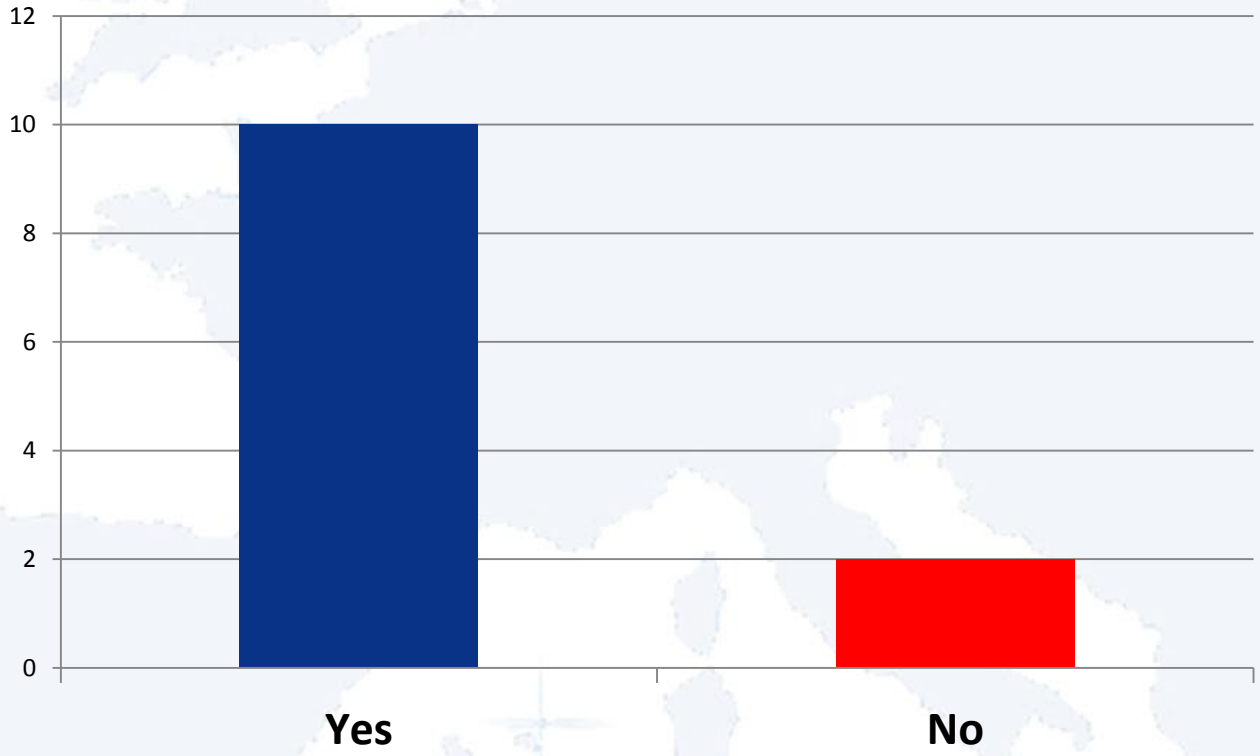
YES (11/13 or 85%) and NO (2/13 or 15%) 1 no answer

Q10. Did you participate in these discussions?

YES (7/11 or 64%) and NO (4/11 or 36%) 3 no answer

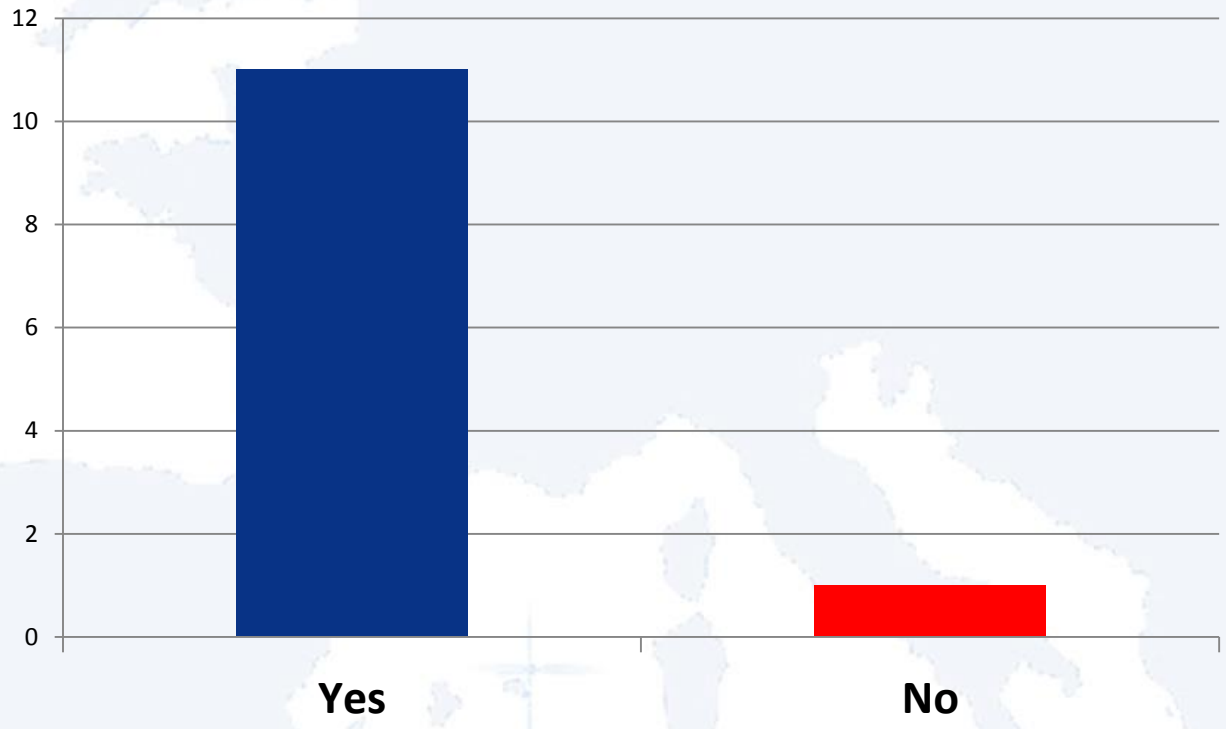
Responses to survey – benefit/risk input

Q12. Was the discussion on benefits an risks constructive?



Responses to survey – benefit/risk input

Q14. Do you feel that you opinion as a patient was (or should have been) an important element of the benefit/risk discussions?



Comments on participation in B/R discussions

- Limited discussion
- Could sometimes give patient view
- Chair very accommodating
- “EMA representative asked if I had input – very helpful”
- Not sure of how serious patient representatives are taken

Responses to survey – benefit/risk input

Q13. Can you describe (within limits of confidentiality) the contribution to the discussions on benefit risk that you made?

- Pros and cons of involvement in research for a particular condition
- Issues around ethics of human trials – raising of hopes for improvement
- Increased risk of heart problems or death –observed in other uses with medicine?
- Product was not temperature stable – concerns about proposed cooling – not practical for patients when travelling

Responses to survey – benefit/risk input

- Combination of medicines – one drug in first part of study resulted in a decline and exacerbation of health markers
- Contribution was my opinion of balance between proposed method of application of drug and severity of the disease – suggested different method of blinding
- Demonstration that patients have different perception of risks to regulators

Conclusions – Suggestions

- Patients need more preparatory information on what is expected from them
- More support – training was mentioned on several occasions
- Earlier contact to ensure enough time
- Address confidentiality issues for patients to discuss with each other.

Suggestions

- Make film as for SAG but directed to SA/PA
- Use testimonies of previous patients
- Post-Summer School webinar planned

- With respect to benefit and risk, opinions were varied
  - Some felt like a piece of the puzzle not a part of the team
  - Some felt confidentiality was too restrictive
  - Some felt clinicians had a role to play in providing an overview for patients to make a decision

- It was clear that all participants would have liked more support and more information

Thank you for your attention

Many thanks to:

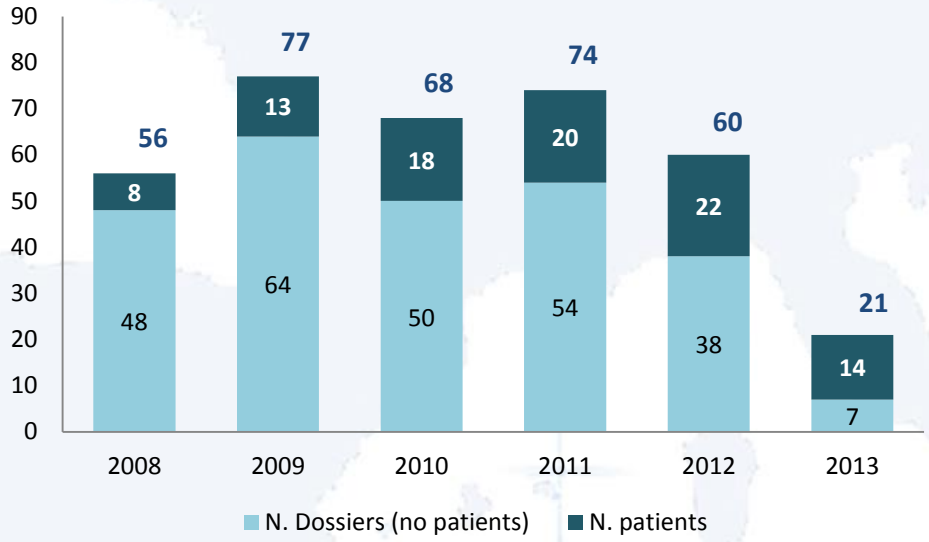
- Nathalie Bere (EMA)

- All the patients who participated in the survey and in Protocol Assistance

- Extra slides

Protocol Assistance – patients involvement

Year	N. Dossiers received by EURORDIS	N. patients
2008	56	8
2009	77	13
2010	68	18
2011	74	20
2012	60	22
2013	21	14



Responses to survey – participation/support

Q1. Had you heard about protocol assistance prior to being contacted to be involved?

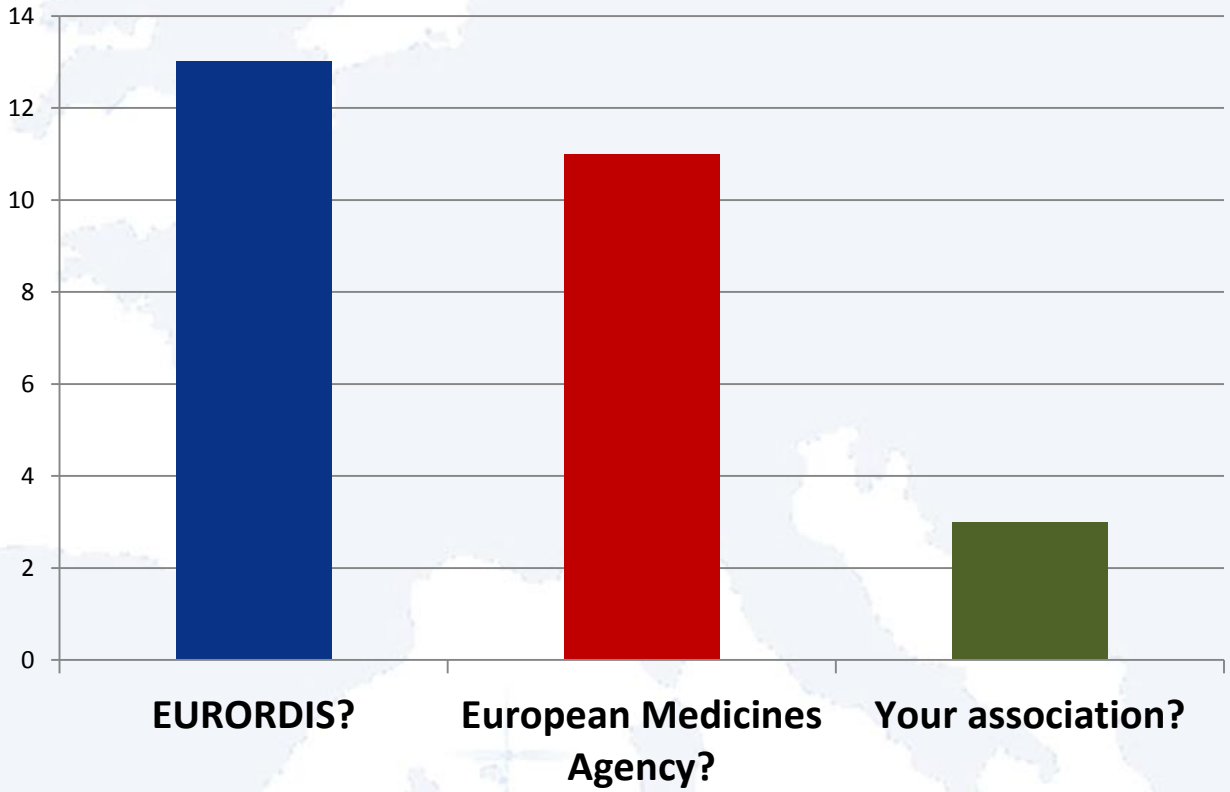
YES (11/14 or 79%) and NO (3/14 or 21%)

Q2. Did you receive enough information, regarding the scientific process and logistics, at the time you were contacted to participate?

YES (11/14 or 79%) and NO (3/14 or 21%)

Responses to survey – participation/support

Q4. Prior to your participation in protocol assistance, did you receive enough support from:



Additional information/support

- Structure and purpose of the briefing packages
- Short description of the goal of the discussion meeting
- Which parts (of the large volume of documents) to focus on
- A focused questionnaire on specific input of patients to benefits and risks
- Clarity on scope of confidentiality – to be able to discuss with other patient representatives
- Longer discussion during the meeting
- Overcome technical problems with passwords
- Feedback from previous participants on procedure, personal input, structure of papers..