EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

Involvement of patients in CHMP OEs; Outcome of Pilot project

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An agency of the European Union

Rationale for the pilot:

- The added value of including patients' perspectives within EMA benefit/risk considerations has been demonstrated many times;
- Patients already involved in benefit-risk evaluations within SAG/ad-hoc expert group meetings, other committee consultations & scientific advice procedures;
- It was felt this could be expanded further within CHMP and provide additional opportunities for patient input;
- In line with CHMP work programme and the Agency's overall emphasis on stakeholder engagement;
- Building on this, a pilot proposed whereby patients invited to participate in B/R discussions at CHMP during specific OEs

Pilot methodology:

- Patients invited to participate where their involvement anticipated to bring added value to the discussion; case-by case basis
- Patients (or carers) selected depending on relevance of their experience/knowledge of particular disease/condition under evaluation; and after assessment of any COI
- Two patients invited, accompanied by a ‘mentor’ (PCWP member); in addition EMA provides personal support (guidance on the work of the EMA/CHMP, the issues for discussion & clear definition of their role)
- Patients give their views and participate in the discussions; including asking questions to the company; they do not take part in decision-making process (leave the room prior to voting).

Pilot duration: Sept 2014 – Nov 2016

1. Sept 2014 - **Scenesse** (afamelanotide) – treatment of erythropoietic protoporphyria (EPP)

2. Jun 2015 - **Intuniv** (guanfacine) – treatment of ADHD in children & adolescents

3. Oct 2015 - **Tecfidera** (dimethyl fumarate) – treatment of multiple sclerosis (Referral procedure related to risk management of PML)

4. May 2016 - **Kyndrisa** (drisapersen) - treatment of Duchenne muscular dystrophy

5. Jun 2016 - **Translarna** (ataluren) - treatment of Duchenne muscular dystrophy

6. Nov 2016 - **Translarna** (ataluren) - treatment of Duchenne muscular dystrophy

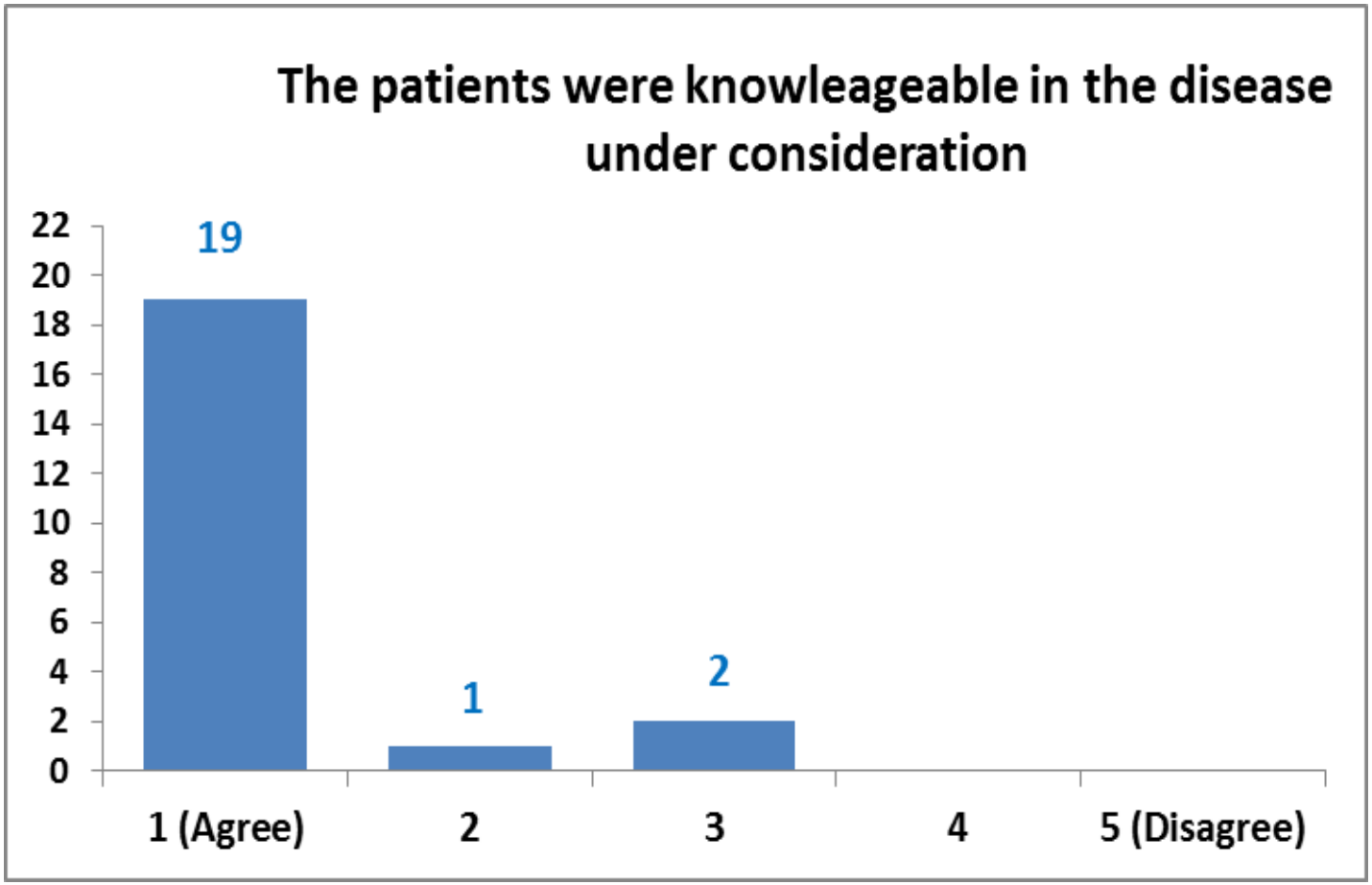
Feedback

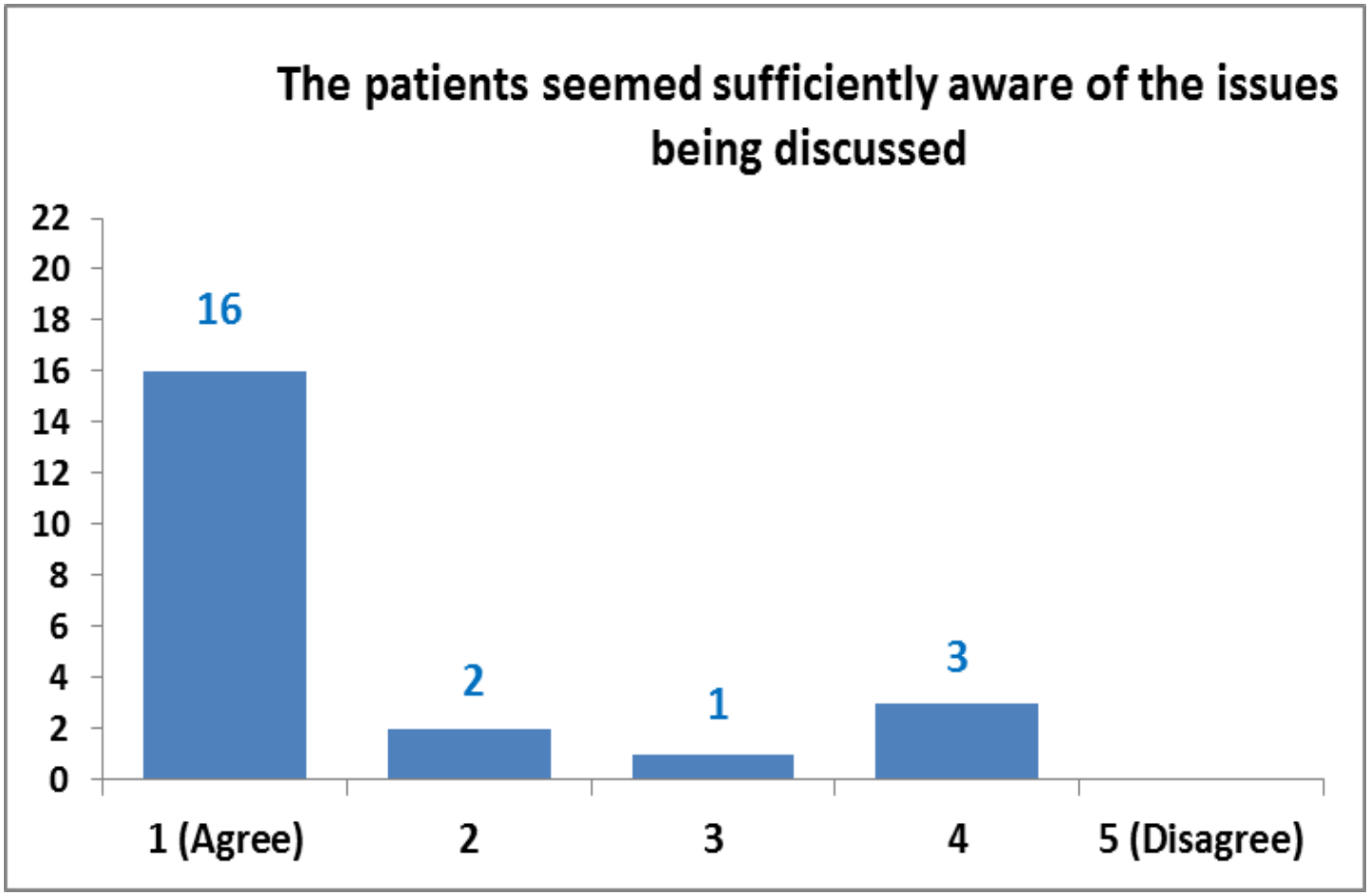
After each case, questionnaires were sent to:

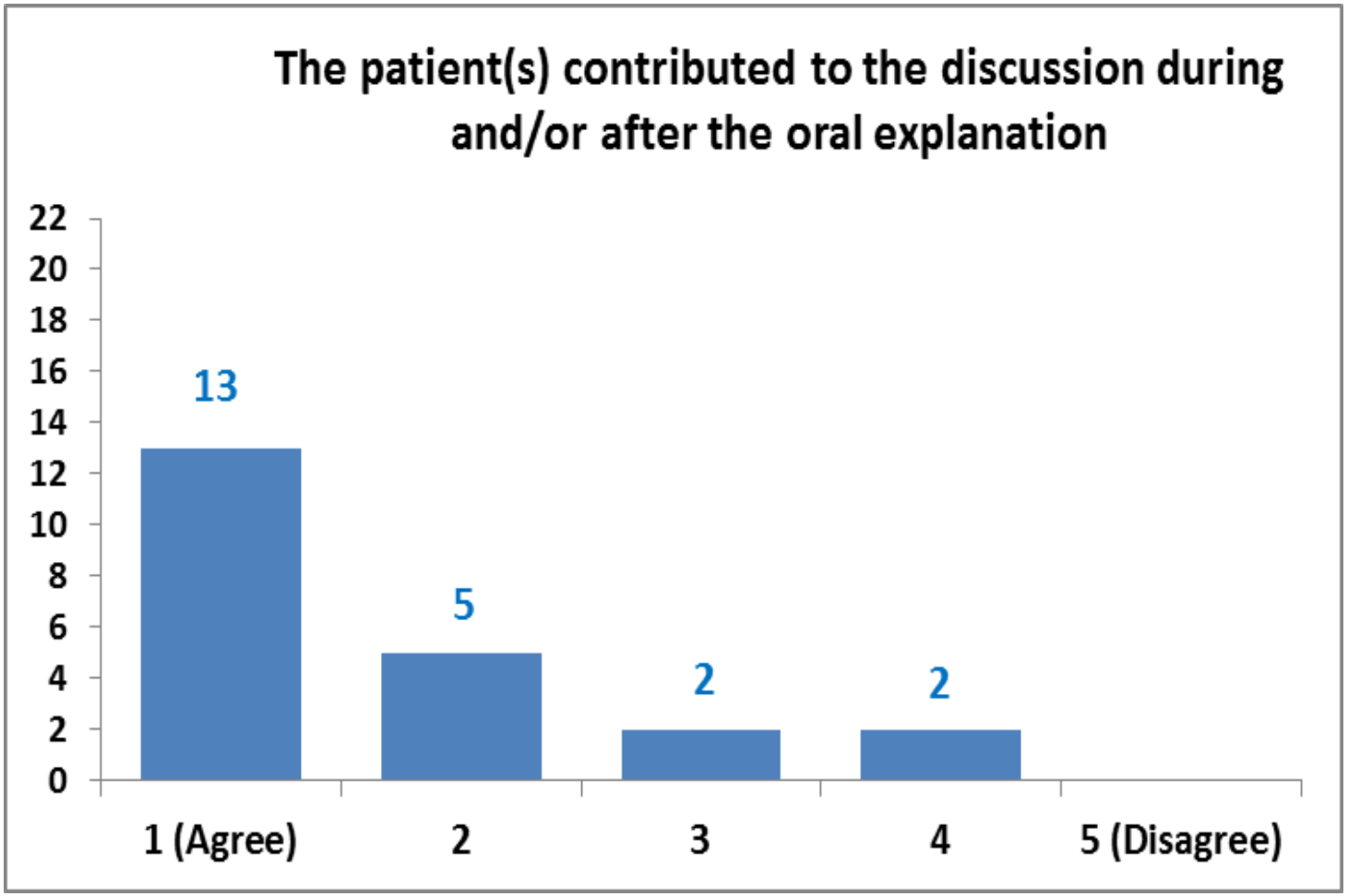
- CHMP working group
- Relevant rapporteurs
- EMA product leaders (EPLs)
- Patients / carers who participated

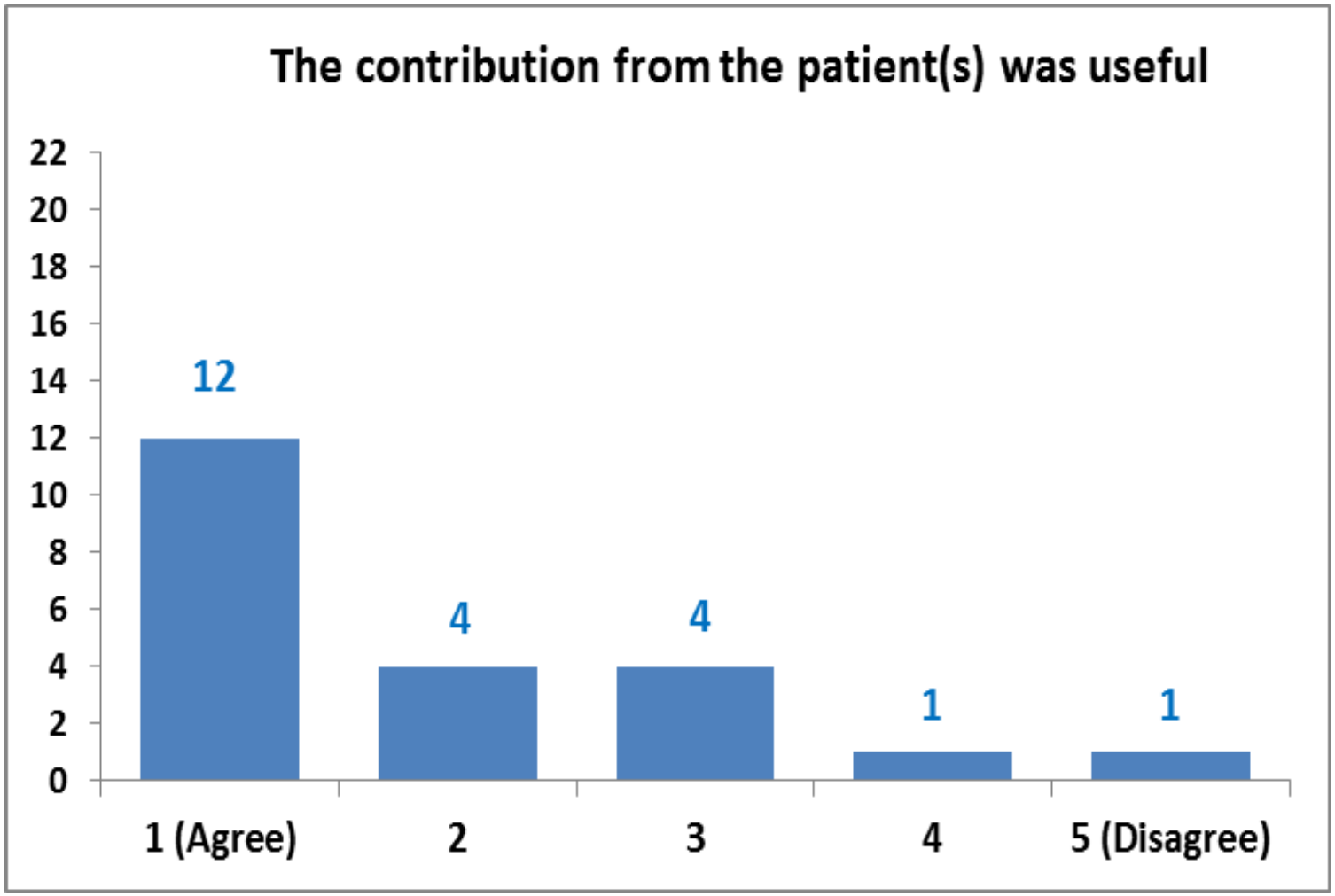
Total of 36 responses received (22 CHMP/EMA, 14 Patients/carers)

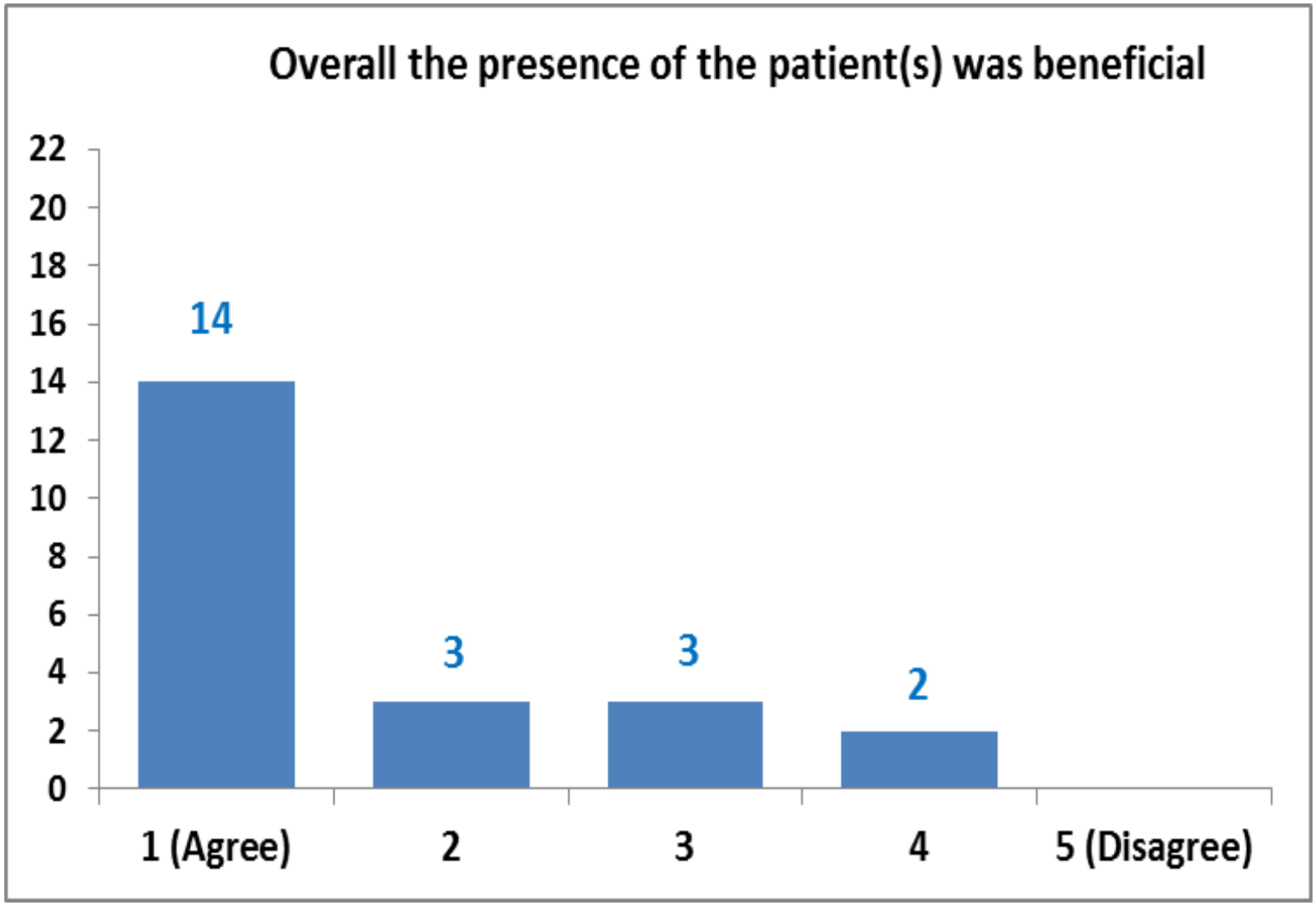
Cumulative responses from CHMP & EPLs



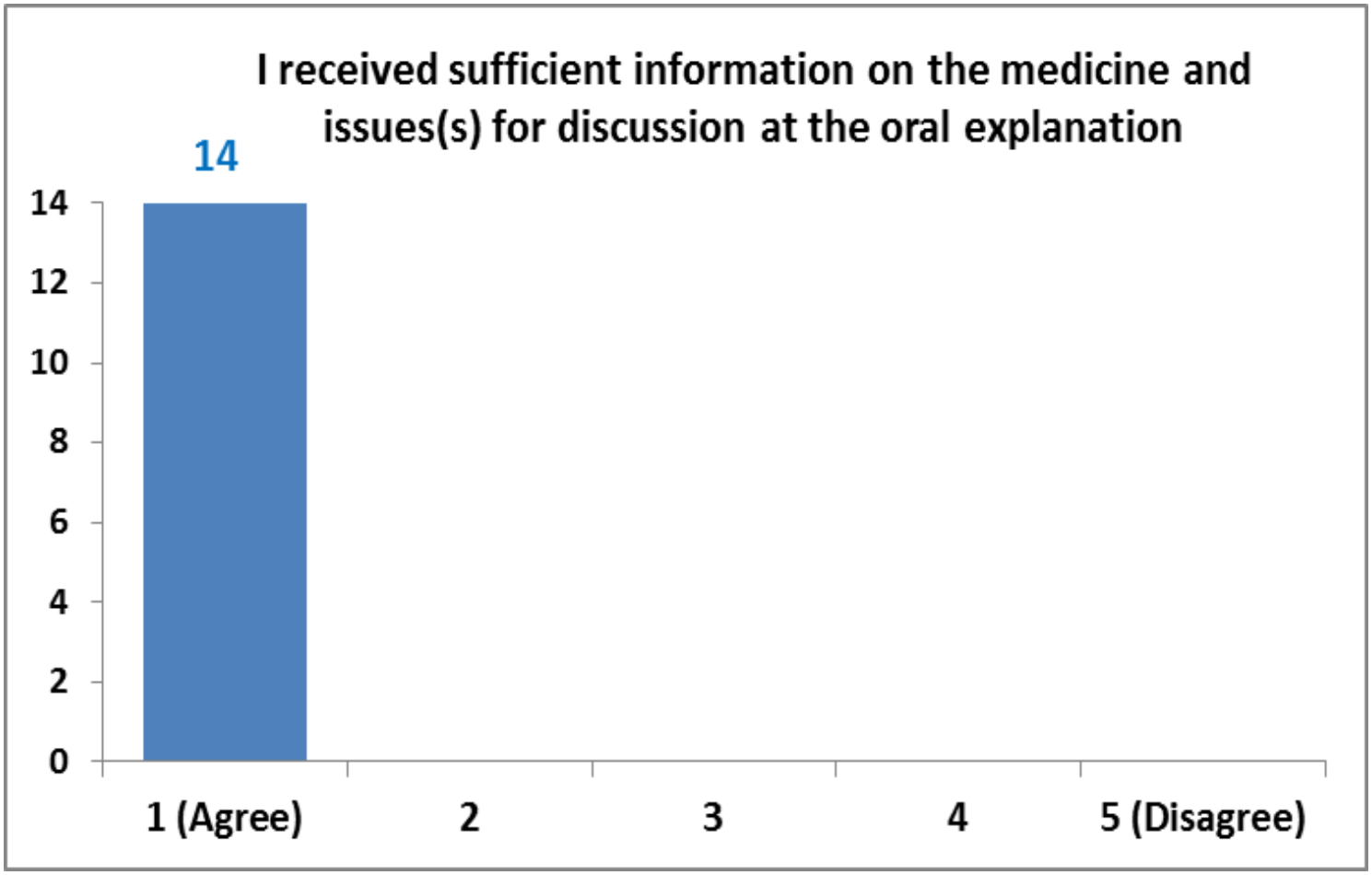

The patients seemed sufficiently aware of the issues being discussed
22
20
18
16
14
12
10
8
6
4
2
0
16
2
1
3
1 (Agree)
2
3
4
5 (Disagree)

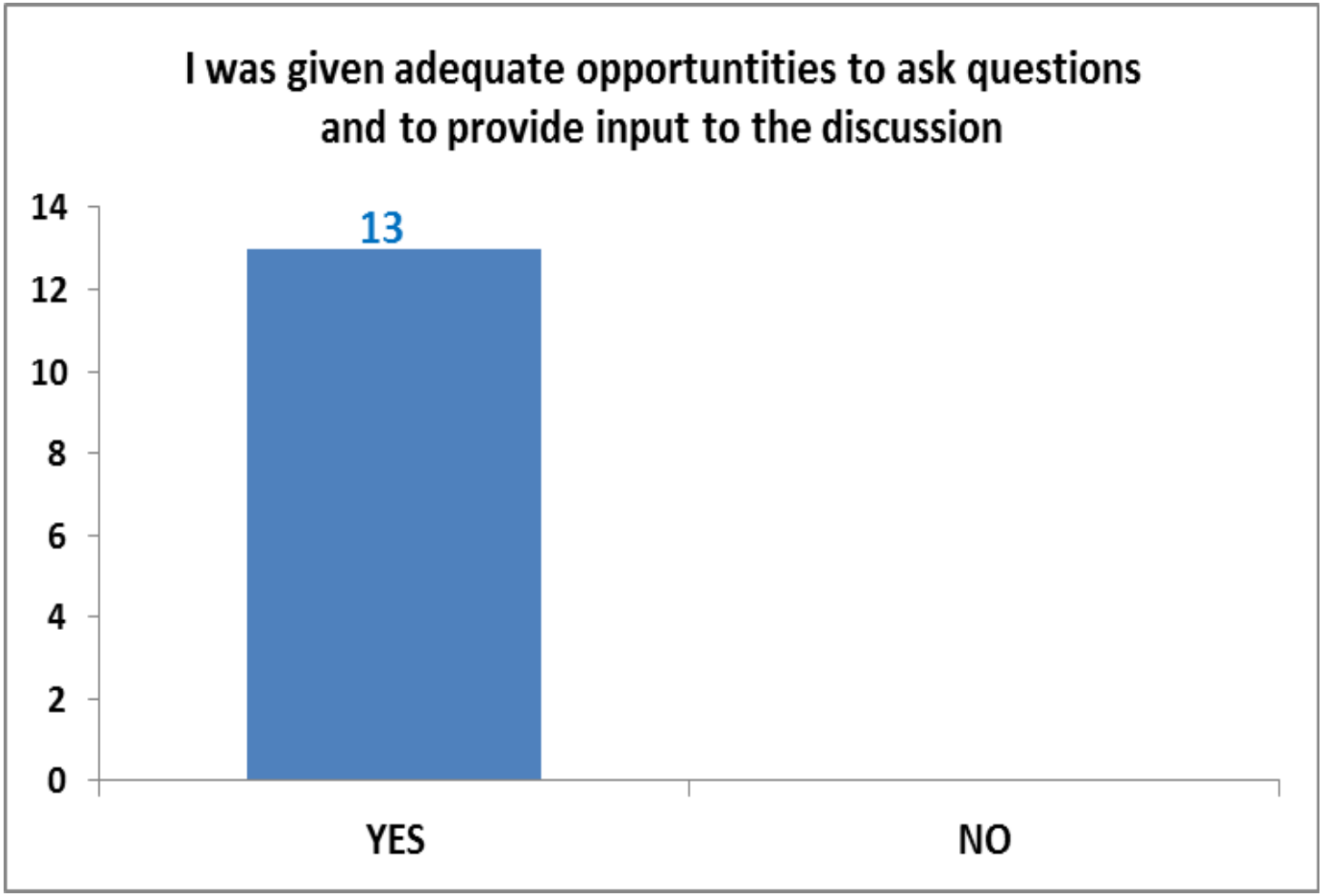

The patient(s) contributed to the discussion during and/or after the oral explanation
22
20
18
16
14
12
10
8
6
4
2
0
13
5
2
2
1 (Agree)
2
3
4
5 (Disagree)

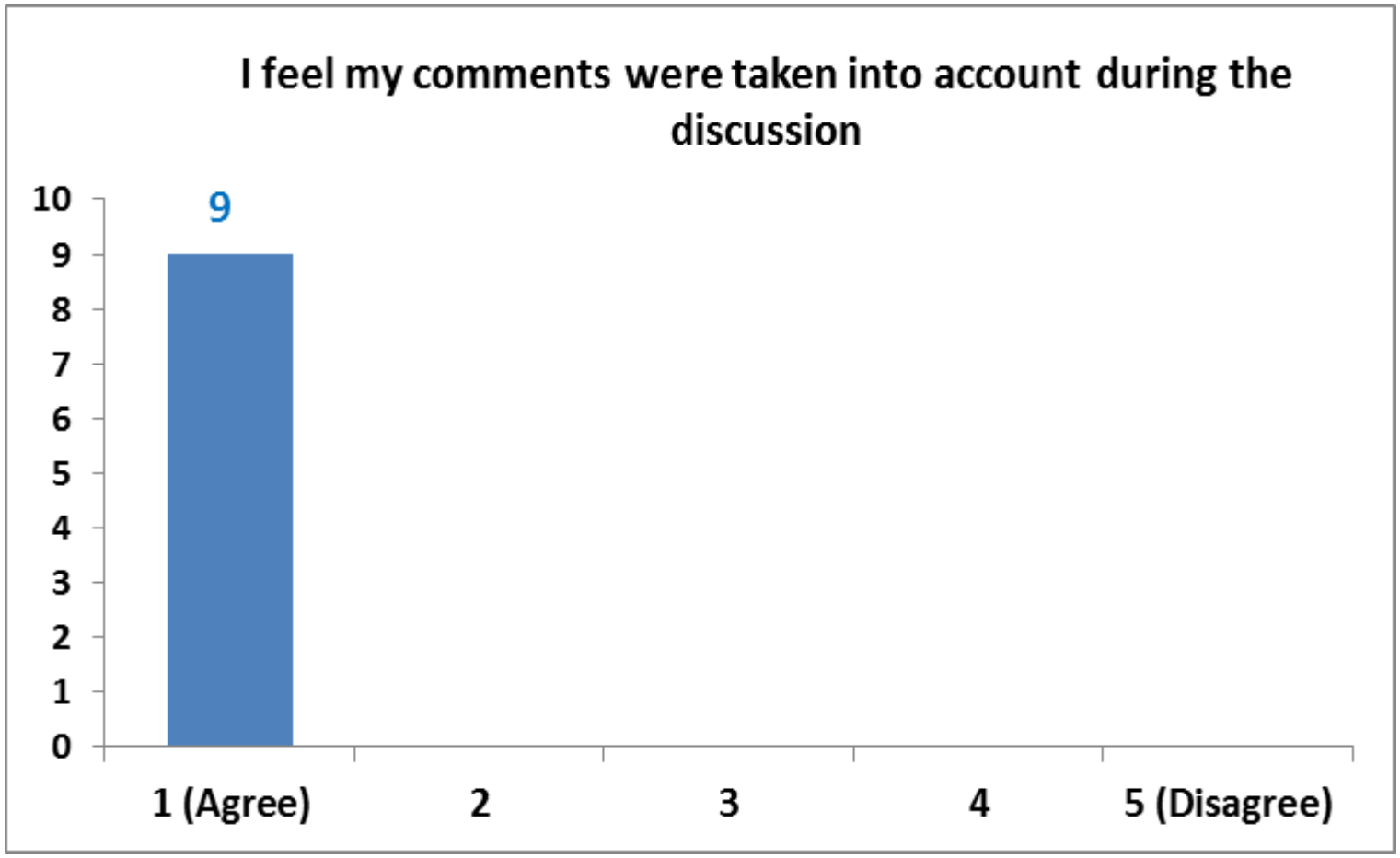

The contribution from the patient(s) was useful
22
20
18
16
14
12
10
8
6
4
2
0
12
4
4
1
1
1 (Agree)
2
3
4
5 (Disagree)

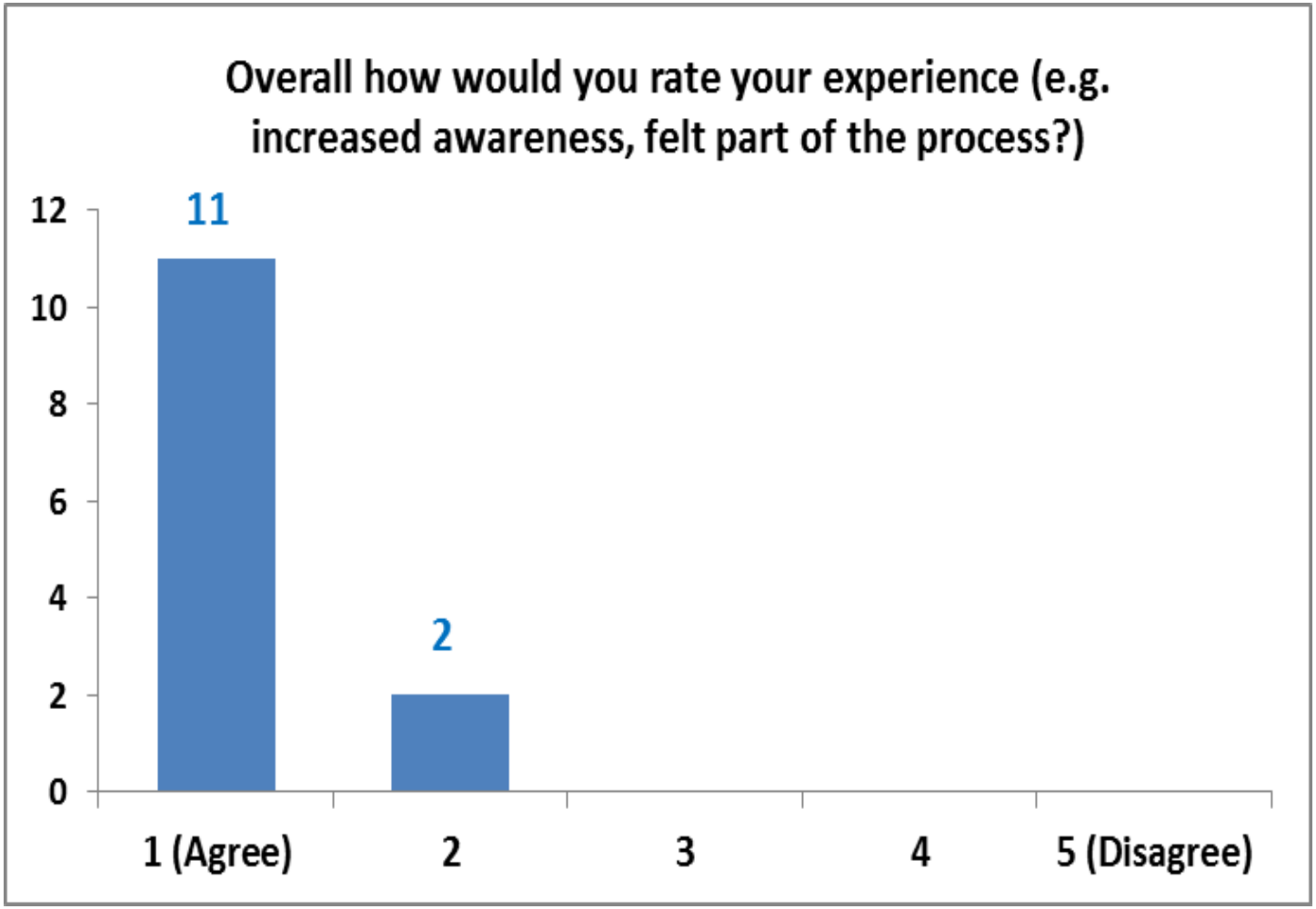

Overall the presence of the patient(s) was beneficial
22
20
18
16
14
12
10
8
6
4
2
0
14
3
3
2
1 (Agree)
2
3
4
5 (Disagree)

Cumulative responses from Patients / carers




I was given adequate opportuntities to ask questions and to provide input to the discussion
14
12
10
8
6
4
2
0
13
YES
NO


I feel my comments were taken into account during the discussion
10
9
8
7
6
5
4
3
2
1
0
9
1 (Agree)
2
3
4
5 (Disagree)


Overall how would you rate your experience (e.g. increased awareness, felt part of the process?)
12
10
8
6
4
2
0
11
2
1 (Agree)
2
3
4
5 (Disagree)

Conclusions from pilot

- Feedback from CHMP/EMA received during pilot is generally positive
- Patients report a very positive experience; increases transparency and trust in the work of the CHMP
- Each case is variable depending on the topic and on patients involved
- Involvement has been a learning curve and has improved with experience
  - More relevant questions for the patients; focused on the assessment
  - Everyone involved knows better what to expect

Proposed way forward

- Continue to invite patients to oral explanations on a case-by-case basis (when input could be valuable to the assessment);
- In addition use alternative methods to consult patients more regularly;
  - Participate in CHMP discussion by TC; respond to specific pre-defined questions, not necessarily limited to OE
  - Written consultation; anytime during evaluation; allows for consultation outside of plenary meetings & usually includes feedback from larger number of patients
  - Elicitation of patient preferences (MCDA methodology currently under investigation)

Thank you - Questions?

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