



3 March 2017
EMA/191955/2017
Stakeholders & Communication Division

Outcome Report on Pilot to involve patients in benefit/risk discussions at CHMP meetings

1. Background/Rationale

A range of mechanisms have been put in place throughout the medicine lifecycle to acquire patients' perspectives within EMA benefit/risk (B/R) considerations and the added value has been demonstrated many times. It was felt however that one area which could be further expanded was within the Committee for Medicinal Products for Human Use (CHMP) meetings to allow additional opportunities to hear and take account of patients' views during the assessment of B/R. This is in line with the CHMP work programme which recommends further integrating patients' values in the B/R assessment and reflects the Agency's emphasis on stakeholder involvement.

2. Involvement in benefit/risk discussions

Patients are involved in B/R evaluations through participation in SAG/ad-hoc expert group meetings, scientific advice procedures and also written consultations. Building on this, it was proposed to invite patients to participate in specific B/R discussions during CHMP meetings, where they would be able to contribute to the CHMP discussions at the time of an oral explanation. It was decided to trial a pilot phase for a period of at least one year to fully assess the feasibility of the proposal and explore how this could occur to maximal effect.

3. Pilot phase

Methodology

Patients were invited to participate during medicine-specific oral explanations where it was felt their involvement could bring added value to the B/R discussion.

The practical aspects of the pilot are described below:

- The Rapporteurs and EMA product leads decided on a case-by-case basis when a specific oral explanation would benefit from the involvement of patients, i.e. when the questions referred to B/R aspects, and in particular,
 - When the CHMP is still undecided on a marketing authorisation application for a new medicinal product in an area where there remains an unmet medical need and would like to assess the impact of their recommendation on the relevant patient population;



- When the PRAC and/or the CHMP would like to assess the impact of their recommendation to maintain, suspend, revoke a marketing authorisation, or to restrict the indication of an authorised medicine, on the relevant patient population.
- Patients were contacted via the EMA network of eligible patient/consumer organisations, other European or national organisations or individuals who have expressed an interest to participate in the Agency's work.
- At least two patients (or carers) with personal experience and knowledge of the particular disease/condition under evaluation were invited to participate. They were selected depending on the relevance of their experience on the topic for discussion and as much as possible from across Europe, bearing in mind that the meetings are conducted in English.
- Each patient completed a Declaration of Interests (DoI) and signed a confidentiality undertaking which was assessed prior to formal invitation, the same as any other invited expert.
- A guidance document was prepared and given to each patient prior to involvement. They also received personalised support from EMA staff members to ensure they understood the work of the EMA and the CHMP, the issues for discussion, as well as a clear definition of their expected role. In addition, specific questions for the patients were sent in advance to be addressed during the OE.
- The patients were also accompanied by a 'mentor'; a member of the Patients and Consumers Working Party (PCWP) experienced in EMA procedures, to provide additional support.
- Patients who participated were invited to share their views and participate actively in the discussions; including the possibility to ask questions to the company. They however did not take part in the final committee members voting process.

4. Outcome results

The pilot ran from September 2014 to December 2016 and included the following oral explanations:

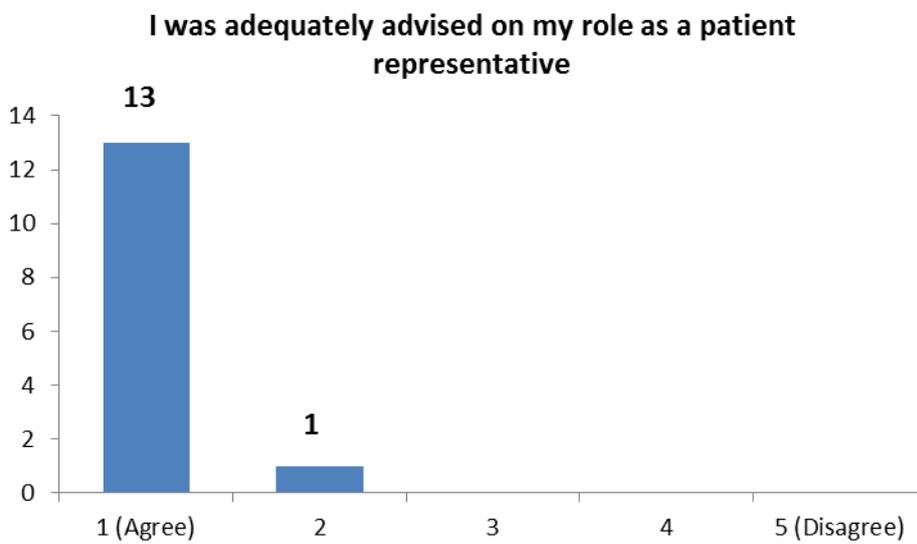
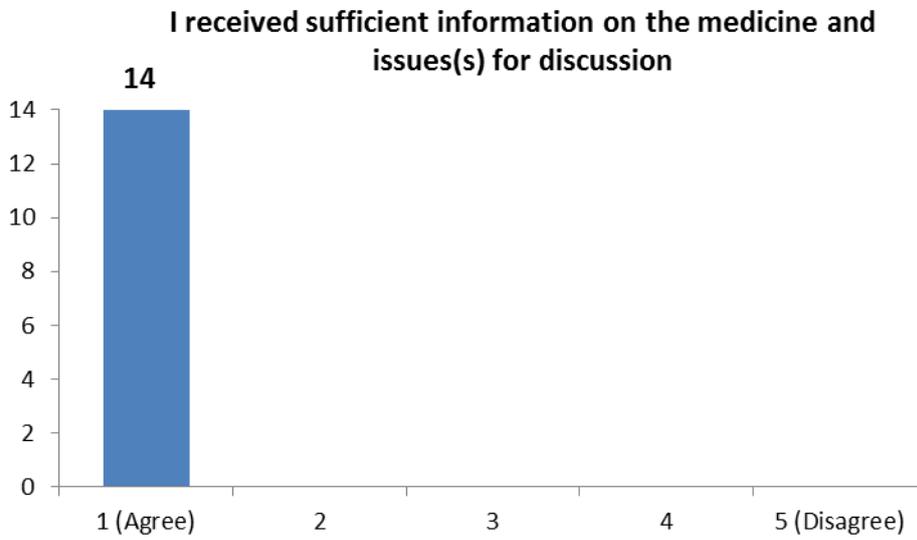
1. September 2014 - Scenesse (afamelanotide) – treatment of erythropoietic protoporphyria (EPP)
2. June 2015 - Intuniv (guanfacine) – treatment of ADHD in children & adolescents
3. October 2015 - Tecfidera (dimethyl fumarate) – treatment of multiple sclerosis (Referral)
4. May 2016 - Kyndrisa (drisapersen) - treatment of Duchenne muscular dystrophy
5. June 2016 - Translarna (ataluren) - treatment of Duchenne muscular dystrophy
6. November 2016 - Translarna (ataluren) - treatment of Duchenne muscular dystrophy

Patient participation and contribution was evaluated by way of questionnaires which were sent after each case to the patients, CHMP members and EMA staff who were involved.

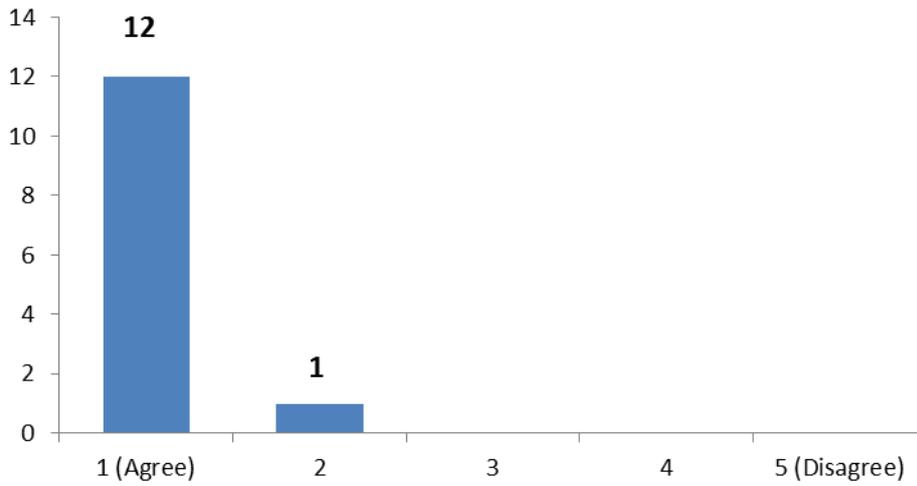
A total of 36 responses were received (14 Patients/carers and 22 CHMP/EMA members) - their feedback and its analyses are the basis for the present report and outcome.

The overall results for each question are shown in the graphs below:

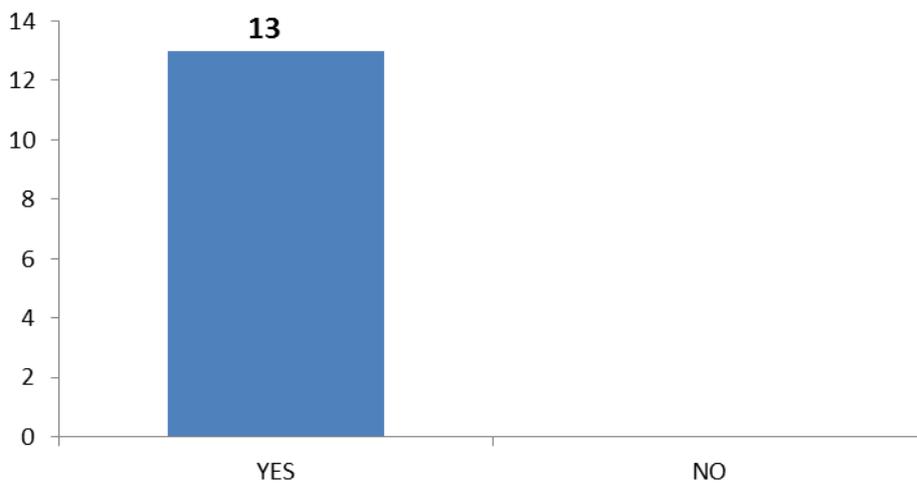
Cumulative responses from Patients / carers



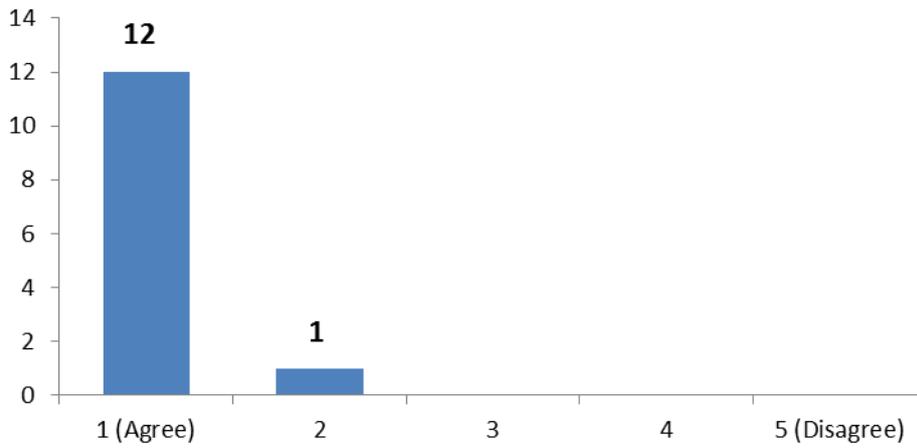
I was able to follow the discussion



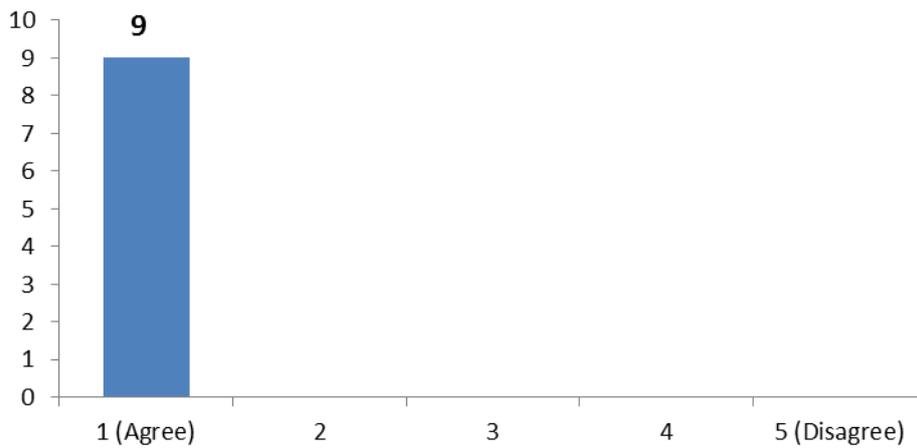
Was the patients view specifically requested?



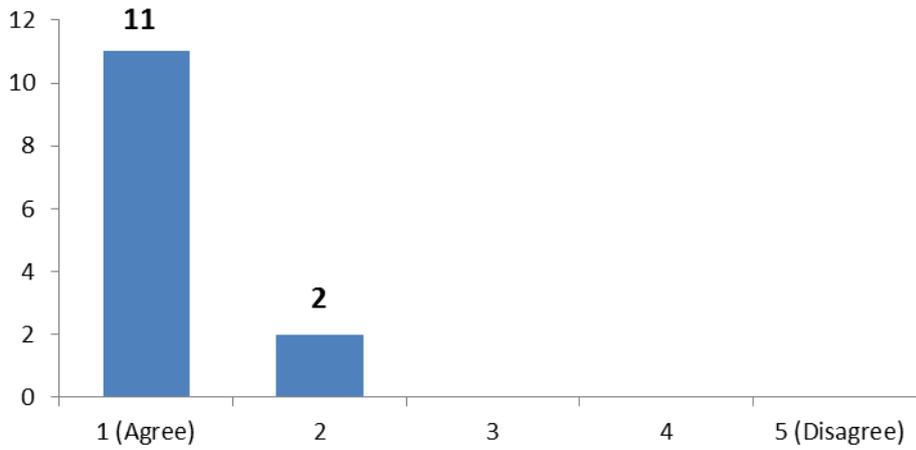
I was given adequate opportunities to ask questions and to provide input to the discussion



I feel my comments were taken into account during the discussion

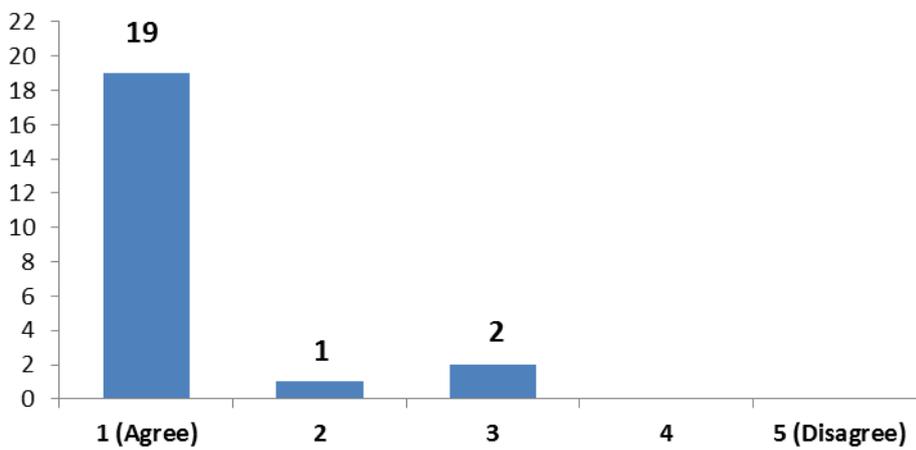


Overall how would you rate your experience (e.g. increased awareness, felt part of the process?)

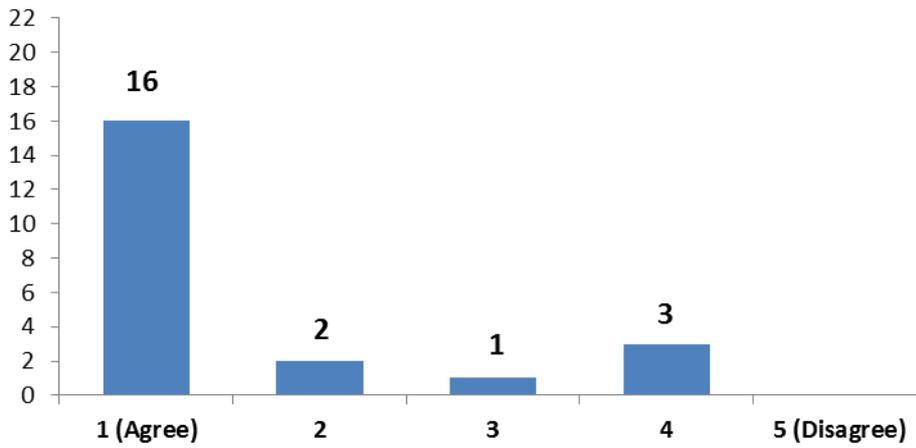


Cumulative responses from CHMP & EMA

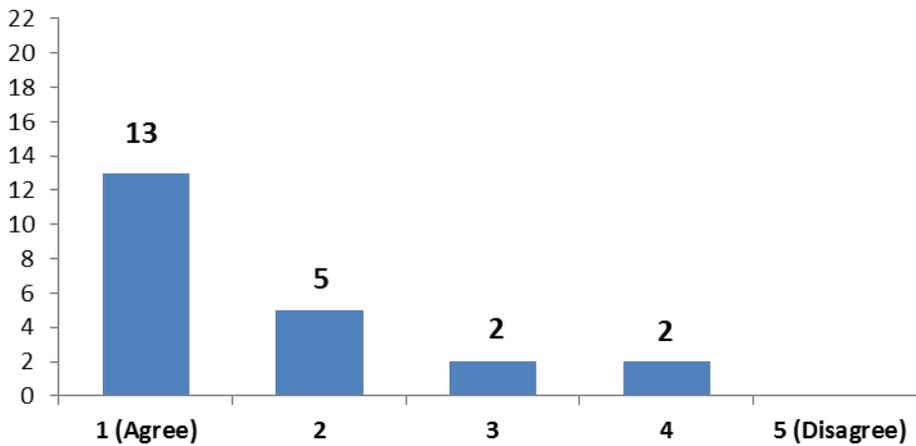
The patients were knowledgeable in the disease under consideration



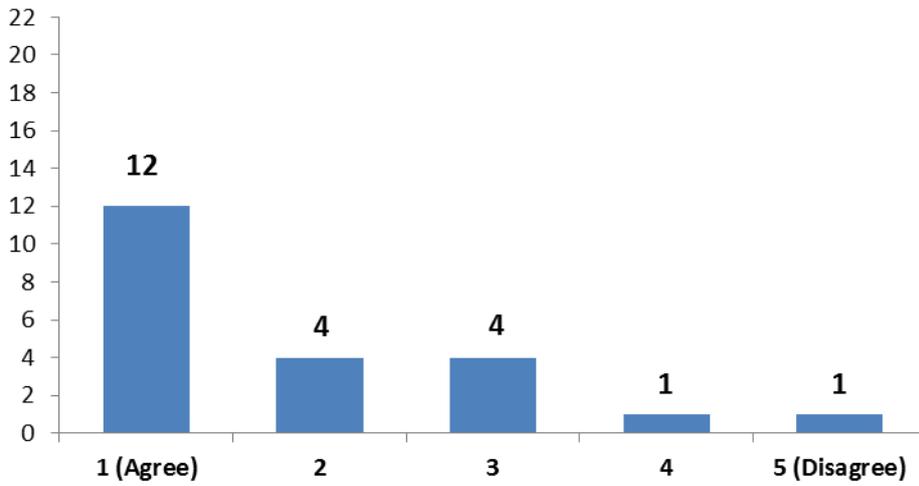
The patients seemed sufficiently aware of the issues being discussed



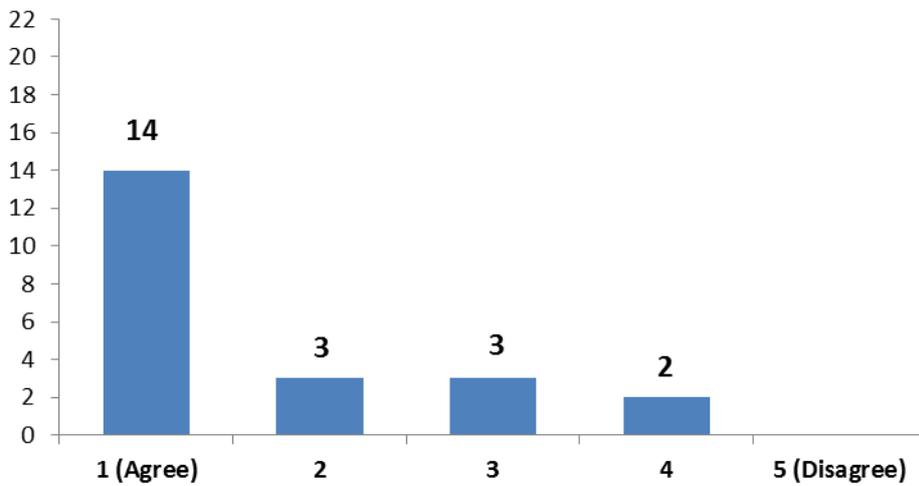
The patient(s) contributed to the discussion during and/or after the oral explanation



The contribution from the patient(s) was useful



Overall the presence of the patient(s) was beneficial



Summary of responses

Feedback from Patients:

Looking at the responses received from the patient participants we can see that they all felt they received sufficient information on the issues to be discussed during the OE and on their expected role and that they were able to follow the discussion and their views were specifically sought. They all also felt that they were given adequate opportunities to contribute and more importantly that their comments had been taken into account.

Overall they gave a very positive feedback on the experience which included feeling part of the process and having a better understanding of the regulatory process regarding the particular medicine under evaluation.

Qualitative feedback received:

"I felt our comments were, despite the fact the patient view was not exactly overlapping with the pure scientific arguments, taken seriously and some CHMP members really tried to include the patients' perspective in their own conclusions. Highly appreciated. We were given ample opportunity to speak/present".

"If this wasn't about my own son it was a totally fascinating and developmental experience. I thought we were listened to as much as possible. However, there are specific issues related to the very complex condition that it is very difficult to capture in any way".

"The participation of patients in this CHMP oral explanation is key for the patient community to understand the regulators' reasoning, for the acceptance of the decision, and for transparency in general".

"A thoroughly worthwhile and encouraging experience from my prospective and hopefully of value to the EMA".

"Extremely well organised. Plenty of opportunity given during the meeting to ask questions and give our views as patient representatives".

Feedback from CHMP and EMA members:

The responses received from the CHMP and EMA staff members can be summarised as follows:

20 out of 22 responders felt that the patients were able to follow the discussions (2 were neutral).

18 members felt that the patients were sufficiently aware of the issues under discussion, with 1 neutral response and 3 slightly disagreeing. Here it is important to bear in mind that much of the discussions are technical and the parents, without medical background, are not expected to follow all of it.

Feedback on whether the patient contributed to the discussion is again generally positive (15 respondents) with 2 slightly disagreeing. Even though all the patients contributed to the discussions in the room, it is natural that some input was more impactful than other contributions.

16 responders felt that the patient representative contribution was useful (72%), however there were 2 responders who felt that it was not so useful and 4 were neutral.

The final, and perhaps most important question "was the overall presence of the patient beneficial" was very positive; 17 agreed (77%), 2 disagreed and 3 were neutral.

We can conclude that the overall feedback received from the CHMP and EMA staff members involved is positive. There are a minority who were not positive. The overall message from the members is that they felt the patients knew the disease under discussion, they actively participated and that this participation was useful.

Qualitative feedback received:

“The patients participating presented two sides of the same problem and it was important to have these different contributions from both of them. The language used and the short statements supporting their concerns were also of a very interesting level”.

“It was very helpful that the patient representatives provided their feedback to the CHMP in the context of the questions that they had been asked”.

“The patients should be involved when there is disagreement between Rapporteurs (on clinical indications) to confirm clinical symptoms concerning B/R to the CHMP Members before their final evaluation”.

“We should ask the patients to focus less on how it is to live with the disease and more on the issues at stake. They need to understand that they to some extent represent the patient community”.

“We must clearly warn patients that once selected to be part of an expert panel to be at CHMP they should not meet the Company or respond to their emails or contact. The fact that during the morning before oral explanation the patient representatives met with the company was very uncomfortable for me. This could have hindered the enormous value of patient’s participation”.

“The two patients attending were both extremely articulate and clearly expressed their experiences on the disease and in relation to using the medicine. It cannot be said that the patients were neutral with regard to the treatment in question - it would have been preferable to have some representation from patients who had not been treated with the medicine and had not already concluded on the benefits of the treatment. The committee spent a long time being told about the unmet medical need, this was not at stake and the duration of time spent on this was, arguably, disproportionate. The contributions of the patients would have been better targeted spending more time on the questions posed by CHMP”.

5. Conclusion

The overall feedback received from both the CHMP/EMA members and the patients involved during the pilot is very positive and reflects the usefulness and benefit of including patients within CHMP discussions when there is an opportunity to enrich the B/R discussions with a patient perspective.

Patients report a very positive experience; they felt listened to and included and this increases transparency and trust in the work of the Agency. The CHMP members felt that it was ‘important’ and ‘very helpful’ to have the patient input during the oral explanation and subsequent discussions.

There was also a learning curve with each case whereby there were improvements as experience was gained, e.g. preparing more focused questions for the patient representatives, and generally everyone involved knew better what to expect.

There is variability to any experts’ participation in any EMA meeting; some discussions lend more to patient input than others, however, it goes without saying that there is an intrinsic value to having patients present and thus enable his/her input when needed.

Way forward

At the end of the pilot phase and the analysis of the feedback received, it was proposed to continue to invite patients to oral explanations on a case-by-case basis (when input could be valuable to the assessment), but also to use additional methods and consult patients on a more regular basis.

This could include participating in CHMP discussions by teleconference or through written consultations at any time during an evaluation (respond to specific pre-defined questions). These options allow for consultations to be conducted outside of plenary meetings and not limited to oral explanations, they also give the opportunity to gather feedback from a larger number of patients, when required. Elicitation of patient preferences is also another patient engagement methodology which the committee and the EMA is currently investigating.

The CHMP members agreed unanimously on the proposed way forward as it is clear that the inclusion of a patient viewpoint enriches the overall evaluation of the benefit and risk of the medicine.

It was also felt that the learnings from this pilot could be shared with other committees, for example the Pharmacovigilance Risk Assessment Committee (PRAC) which also evaluates B/R, with a view to making more use of this mechanism during such assessments.

This is a very important milestone in completing the range of opportunities for patient engagement along the medicine lifecycle. The CHMP (due to legislation) does not have civil society members, and this new opportunity will allow the committee to gather information directly from patients whenever needed.