EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

13 December 2013
EMA/272219/2013
Stakeholders and Communication Division

Sixth annual report on the interaction with patients' and consumers' organisations (2012)

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Executive summary

Introduction

Patients' representatives are involved in many different activities at the EMA and as such are included in discussions leading to agency outputs. The Agency's interaction with patients and the organisations that represent them, has long proven extremely beneficial; they make available important perceptions on the use of medicines and provide valuable insights, not necessarily obvious to the scientific experts. Engaging with these stakeholders delivers an extra dimension and builds a more rounded evaluation.

This report, prepared annually, provides a detailed summary of patients' and consumers' involvement, in this case, during 2012. This also includes the work carried out by the "EMA Human Scientific Committees' Working Party with Patients' and Consumers' Organisations" (PCWP).

A comparison to preceding years is given, potential areas for enhancement are proposed and future activities outlined.

An analysis on the level of satisfaction of patients and consumers involved in the EMA's work during 2012 is also presented.

The current report was presented to the PCWP during its meeting on 6 June and to the Management Board during its meeting on 3 October 2013.

Herewith attached is a link to the EMA glossary of acronym definitions.

Interaction with patients and consumers during 2012

During 2012 a mutually-beneficial collaboration has again been achieved, which has once more increased significantly compared to previous years; from 423 patients and consumers involved during 2011 to 525 during 2012. It is however anticipated that the volume of involvement will reach a plateau in the next year or so.

Patients and consumers have been involved in a wide range of activities at the Agency. It is particularly interesting to note their increased involvement in the benefit/risk evaluation of medicines through the Scientific Advisory Groups, CHMP consultation and Scientific Advice/Protocol Assistance where they bring the most added value in relation to their real life experience of the disease and treatment. They have continued to review material prepared for the public (package leaflets, EPAR summaries, and safety communications). They have also joined numerous conferences and workshops, often as speakers, and they are members of the EMA Management Board, the EMA Committees and Working Parties.

In addition the EMA is also engaged in research activities to explore methods for eliciting patient preferences for use in benefit-risk assessment. This work is being carried out in collaboration with several academic institutions and under the support of the IMI-PROTECT project.

The PCWP has continued to act as a vital platform for exchange between the Agency and patients' and consumers' organisations, on a wide-range of topics of common interest.

Work has continued on the revision of the framework of interaction, to further enhance the formal collaboration between the EMA and patients' and consumers' organisations.

Next steps

As mentioned above, the "framework of interaction" between the EMA and patients' and consumers' organisations will be revised, focusing on three pillars: 1) the role of patients within the scientific committees (document adopted and published, 2) a defined strategy for training and support including a dedicated training webpage and a training video (finalised during 2013), and 3) patients' participation in benefit/risk evaluations (to be finalised).

Patients' and consumers' organisations representatives will continue to be involved in the various activities across the Agency, as detailed within this report, with involvement in benefit/risk evaluations being enhanced where possible.

Due to the success of the involvement of patients' representatives in scientific advice procedures for orphan medicines, this will now be extended to include involvement in scientific advice procedures for other medicines, as of January 2013.

The EMA together with the PCWP will continue looking at its procedures for evaluating the appropriateness of eligible organisations; especially how to handle potential conflicts of organisations when they work with the Agency.

Training will be provided in line with the newly adopted training strategy.

The EMA will look to enhance the concept of public engagement within the framework of interaction, which so far has focused on patients' and consumers' organisations. This will ensure the Agency can meet future challenges, such as public hearings. This enhancement would include an increased network of patients to ensure adequate numbers and flexibility providing access to the most appropriate patients when needed.

The management board will be presented with the next annual report on the interaction with patients' and consumers' organisations in 2014.

The current report is divided in two sections:

Section 1: Overview of patients' and consumers' involvement in EMA activities during 2012.

Section 2: Analyses of the degree of satisfaction of patients and consumers involved in EMA activities during 2012.

SECTION 1:
Overview of patients' and consumers' involvement in EMA activities during 2012

1. Activities involving patients and consumers during 2012

Patients are included as formal members in many of the Agency's Scientific Committees (COMP, PDCO, CAT & PRAC).

There is an increasing involvement of patients in benefit / risk evaluations, specifically via scientific advisory groups (SAGs) where patients having experience of the specific disease/condition under discussion have provided valuable information which has contributed to the product-specific benefit-risk deliberations.

Similarly patients have participated in EMA scientific advice procedures for orphan medicines by providing input on the advice that the Agency gives to the pharmaceutical companies, typically on their clinical development programs (e.g. feasibility/duration of studies, relevant patient population and outcomes, safety concerns).

Patients' representatives are systematically involved in the preparation of the Agency's safety communications, as well as all package leaflets and EPAR summaries for new medicines and those at the time of the 5 year renewal. They review the readability of this information to ensure that it is clear and understandable for patients and the general public.

They also continue to be involved in several on-going EU-wide initiatives, such as EudraCT (EU clinical trials register), Eudravigilance (public database), ENCEPP (European Network of Centres for Pharmacoepidemiology and Pharmacovigilance) and Enpr-EMA (European Network of Paediatric Research).

The Agency has also consulted patients on key aspects related to the new pharmacovigilance legislation, through dedicated meetings with stakeholders as well as direct consultations; e.g. the ADR website, the black symbol and text to be included in the SmPC and package leaflet for medicinal products subject to additional monitoring (see tables below for full list of activities).

The Agency has a well-established 'network of experts' from patients' and consumers' organisations and with their help the EMA was able to reach patients' representatives in many diverse therapeutic areas. This network is an extremely valuable resource which needs to continuously expand due to the increasing number of areas and activities requiring patients' input. An up-to-date list of eligible organisations is published on the Agency website.

The value of the contributions brought to the Agency by 'lay experts' and the unique insights that they share is by now well acknowledged across the different areas and activities in which they are involved.

1.1. EMA Management Board (MB)

The MB includes two representatives of patients' organisations within its membership, appointed by the European Council. The patients' representatives' mandate ended in 2012 with the new members being appointed in 2013.

1.2. EMA scientific committees

Patients are formal members of four EMA Scientific Committees; the COMP, the CAT, the PDCO (and the PRAC since 2013). In addition, all the Committees may consult PCOs on specific issues as and when needed.

Pharmacovigilance Risk Assessment Committee (PRAC)

The new Pharmacovigilance Committee started to operate in July 2012, however patients and healthcare professionals were not nominated as members/alternates until 2013.

Committee for Orphan Medicinal Products (COMP)

The COMP includes in its membership “three members nominated by the Commission”

Two other patients’ representatives were consulted during 2012 as experts in procedures concerning orphan designations.

Paediatric Committee (PDCO)

The PDCO includes in its membership “three members and three alternates appointed by the Commission”.

Two patients’ organisations were also consulted during 2012 as experts in procedures concerning PIP procedures.

Committee for Advanced Therapies (CAT)

The CAT includes in its membership “two members and two alternates appointed by the Commission”. During 2012 there were no patient representative members. New members will be selected and appointed during 2013.

Committee on Herbal Medicinal Products (HMPC)

There is currently no legal basis in Community legislation for patients to be members of this Committee, although interaction is possible through provisions in Article 78 (2) of Regulation (EC) Nº 726/2004.

During 2012 the HMPC consulted with PCOs to obtain feedback on package leaflet advice to be included on the preparation of herbal teas.

From 2013 PCO representatives with a specific interest/expertise in herbal medicines will contribute to the preparation of summaries of herbal monographs which will be published.

Committee for Medicinal Products for Human Use (CHMP)

There is currently no legal basis for patients to be members of this committee. Interaction with the CHMP, its working parties and scientific advisory groups (SAGs) is based on Article 78(2) of Regulation (EC) Nº 726/2004.

During 2012, the CHMP consulted with PCOs to obtain their viewpoint with regard to new diabetes medicines under evaluation. The consultations related to a new insulin strength and the proposed product information and pack designed to minimise the potential of medication errors. The organisations were asked to respond to a list of questions adopted by the Committee and the feedback was then taken into consideration by the CHMP during their evaluation process.

Patients and consumers also participated in an ad-hoc expert meeting to further discuss these issues.

The Agency also met with Thalidomide patients and victims organisations regarding a new thalidomide application for a medicine proposed for the treatment of multiple myeloma. The consultation was held

to obtain their input on the preparation of the risk management plans and the information to be included within the package leaflet.

Representatives of the MB and the Committees are invited to participate in the PCWP meetings as observers to maintain a link between the different groups.

Participation in Scientific Advisory Group (SAG) / ad-hoc expert group meetings

SAGs are groups of European experts convened by the CHMP to provide advice during the evaluation of a specific medicine. Following a pilot phase, and agreement by the CHMP, patients are invited to participate in all SAG meetings.

During 2012, a total of 36 patients participated in 25 different SAG/expert meetings, providing their views on specific questions posed by the CHMP.

The areas covered were: diabetes, HIV infection, cardiovascular, anxiety, multiple sclerosis, renal cell carcinoma, peripheral T-cell lymphoma, burns, axial spondyloarthritis, hepatitis B, Hodgkin lymphoma, short bowel syndrome, obesity, lipoprotein lipase deficiency, anti-infectives, haematopoietic stem cell transplantation therapy, cervical cancer vaccine, Leber's Hereditary Optic Neuropathy, schizophrenia/bipolar disorder, bone disorders, alcohol dependence, small pox vaccine and migraine.

Participation in EMA Scientific Advice (SA) procedures

19 patients' representatives participated as experts in specific scientific advice requests from pharmaceutical companies for orphan drugs (protocol assistance). Representatives give their input during attendance at a SAWP plenary meeting or by submitting comments in writing.

As the range of therapeutic areas discussed within SAG and SA meetings is wide, it is sometimes a challenge to locate patients with experience of the particular condition/disease under discussion.

Participation in the Pharmacovigilance Working Party (PhVWP)

Up to June 2012 patients' and consumers' representatives participated in the PhVWP as observers; one patient representative and one alternate until the creation of the Pharmacovigilance Risk Assessment Committee in July 2012.

1.3. EMA Working Party with Patients' and Consumers' Organisations (PCWP)

The PCWP continues to play a key role as a platform for exchange of information between the EMA and PCOs. The composition of the PCWP during 2012 was as follows:

- 15 members and 13 alternates representing PCOs;
- 5 members from the EMA Scientific Committees (CHMP, COMP, PDCO, HMPC & CAT);
- 1 member from the EMA secretariat;
- Observers from CMD-h, HCP WG, PhVWP and the EMA management board.

There were four PCWP meetings held during 2012, including one meeting with all 'eligible' organisations, and two joint meetings with the Healthcare Professionals Working Group (HCP WG). In addition, a one-day training session for all experts was held in November.

1.4. Activities related to implementation of the new pharmacovigilance legislation

Stakeholder meetings

The objective of these meetings is to raise awareness of the requirements of the new legislation and to provide a forum for exchange of concerns and opinions. Three stakeholder forums took place during 2012 and included participants from patients' and consumers' organisations. These participants also joined a briefing teleconference before each of the meetings.

The first meeting, held in February, presented an update on the implementation process, transitional arrangements and discussed the good pharmacovigilance practice (GVP) modules that have been released for public consultation. Nineteen patients' representatives participated.

The second forum, held in May, presented an update on the implementation process and gave the opportunity for stakeholders to feedback from recent consultations, including questions and answers on the transitional period. Six patients' representatives participated.

The third meeting was held November; it presented an update on the prioritised implementation and future planning, shared first experiences on activities undertaken at EMA level and obtained feedback from stakeholders on the overall implementation process. Twelve patients' representatives participated. The European Aids Treatment Group gave a presentation on the "Patient experience to date".

Stakeholder meetings will continue to take place during 2013 and patients' representatives will be invited to participate and to provide their input.

Additional monitoring of medicines & direct patient reporting - impact on the package leaflet

New legislation defines that certain medicinal products are included on a list for 'additional monitoring'. For these products, a black symbol and an explanatory sentence will be included in the summary of the product characteristics (SmPC) and in the package leaflet. Additionally a standardised sentence will also be included in the product information for all medicines, encouraging the reporting of suspected adverse reactions for these particular medicines.

During 2012 the EMA worked on draft proposals prepared by the Quality Review of documents (QRD) in collaboration with the EU regulatory network. The proposals were discussed at the PCWP meeting in September. Following this consultation, one PCWP member then presented the PCWP final position to the Pharmacovigilance Risk Assessment Committee (PRAC) meeting in October for their consideration. On this basis the PRAC issued a recommendation to the European Commission.

Risk management plan summaries (RMP)

The new legislation states that a Risk Management Plan (RMP) is required for all new medicines and that a summary of the RMP should be made public.

In March 2012, following a call for expression of interest, several PCWP members participated in a workshop to help identify key information to be included a summary, the most suitable format and also the discuss the best way for PCOs to be involved in the preparation of individual summaries.

Patients and consumers will continue to be involved in the development of this new type of document and in the review procedure, once it has been established.

1.5. Activities related to clinical trials

The EU clinical trials register

All clinical trials carried out within the EU since 2004 have to be registered in the EudraCT database. In March 2011 this information was made available to the public via the Clinical Trials Register (in accordance with EU Regulation Nos. 726/2004 and 1901/2006).

PCOs have been very involved in the development of the register; 10 representatives have been members of the EudraCT joint operational group since 2010. The group met four times during 2012 to focus on the new public interface to ensure 'user-friendliness'. This included preparation of a guidance document for users and practical user-testing of the system prior to its launch.

The Agency will continue to work with this group to improve the functioning of the register. Plans for the future include the publication of summaries of clinical trial results, which will require a major upgrade to the existing system.

Workshop on access to CT data and transparency

In November 2012 a workshop was held to discuss how to publish clinical-trial data and was the first step in the process to proactive publication of clinical-trial data. Patients' representatives were asked to give their views during the workshop.

There are practical issues and considerations that need to be addressed and the workshop was able to gather the views and concerns from a broad range of institutions, groups and individuals.

Advisory groups with broad representation from all parties, including patients, will be formed and will start working early 2013. The proactive publication of clinical-trial data is expected to come into force on 1 January 2014.

1.6. Activities related to the preparation of information for patients and the general public

All new and renewal package leaflets (PLs), EPAR summaries and safety communications are systematically sent to patients' and consumers' organisations for review. The purpose of the review is to check that the information has been prepared in a 'patient friendly' manner and that the language is appropriate for the target audience.

Eligible organisations nominate experts to review such documents, who are then invited to attend a training session on the review procedure organised by the EMA. There is also a 'training manual' available for perusal on the EMA website.

Experience acquired so far confirms the relevance of comments received with an average 30-50% of comments taken on board. The patients' and consumers' contribution helps improve the quality of the documents within the scope of this procedure.

During 2012, patients' and consumer's organisations' representatives reviewed a total of 102 package leaflets, 36 EPAR summaries, and 24 safety communications.

1.7. Other activities

Involvement in training activities

- **Annual EMA Training session for patients and consumers involved in EMA work**

The annual training session for 2012 consisted of an in depth description of the different elements related to the implementation of the new Pharmacovigilance legislation, specifically those topics of most interest for patients. The session also included an overview of the EMA procedure for evaluating medicines (centralised procedure).

All eligible organisations and their nominated experts are invited to attend and in this case 21 “patients’ experts”, attended the training session.

- **Other training sessions**

As mentioned by Eurordis, another mode of interaction between EMA and PCOs relates to the participation in mutual training activities; not only those that take place at the EMA, but also those organised by PCOs. For example the Eurordis Summer School on EU regulatory affairs where members of the EMA scientific committees and EMA staff are presenters (since 2008), creating an interactive exchange of knowledge and views in exercises such as the "mini-COMP" or "mini-PDCO" sessions, or learning how to review EMA information for the public such as package leaflets. As of 2013, some 140 patients' advocates have benefited from this interaction.

Other EMA consultations

- **EMA survey on communication practices during a pandemic influenza crisis**

In April 2012 the EMA carried out a survey among patients’ and consumers’ organisations to collect input on aspects related with its communication practices during a pandemic influenza crisis (e.g. 2009 H1N1 pandemic).

The outcome of the survey, and subsequent discussion with patients’ and healthcare professionals’ organisations, identified the need for a core group of organisations, with particular capacities (e.g. rapid response to urgent requests) to work closely with the Agency in preparedness activities and in the event of a pandemic. These discussions also contributed to the revision of the Agency’s pandemic communication plan.

The outcome and actions were presented at the PCWP meeting in September 2012 and also to the pandemic team within the EMA.

- **Focus group on emerging information to patients published by the EMA**

A focus group was convened to brainstorm and obtain feedback on the different information that the Agency publishes on its website (e.g. safety communications, press releases). A teleconference was organised to collect the views from patients’ and consumers’ organisations.

The new pharmacovigilance legislation will lead to the publication of new information so it was felt that this would be a good opportunity to review existing information, tools and practices, and identify any areas for improvement.

The feedback received indicated that the amount of information provided by the EMA is sufficient and of good quality, however targeting better this information with the use of new tools able to meet such demands was suggested, and increasing accessibility would be welcomed.

- **EMA survey on communication documents**

Following earlier discussions on EMA communications, it was decided to carry out a larger scale survey to specifically capture the quality and dissemination of question-and-answer documents and press releases produced by the EMA to provide new information about medicines.

Twenty-seven organisations responded and an overview of the results was presented at the joint PCWP/HCPWP meeting in September. In general, the feedback received showed that patients', consumers' and healthcare professionals' organisations were satisfied with the outputs produced by the Agency, but that there was room for improvement. The Agency encouraged the organisations' to disseminate information and messages as much as possible and will work together with these organisations to ensure that Agency information is referenced to its full potential.

- **Review of Q&A on biosimilar medicines**

During 2012 the Q&A on biosimilar medicines was reviewed following feedback from various stakeholders; patients and consumers provided input on the revision.

Involvement in EMA meetings, workshops, conferences and info sessions

- **Working group meeting on funding and COI**

An ad-hoc group meeting was held in February 2012 following previous discussions with the PCWP on how to improve the way to handle possible conflicts of interest of PCOs.

The group presented the outcome of their discussions to the PCWP at their meeting in May 2012, and it was agreed that the proposed document on how to handle funding of organisations should be modified and further discussed. The updated document was discussed at the PCWP meetings in September and November. The document will be re-discussed in 2013.

- **Advanced therapies Workshop**

This one-day workshop was organised by the Committee for Advanced Therapies (CAT) as part of its effort to strengthen dialogue with its stakeholders. The main focus was to communicate the outcome of focus group meetings held in 2011. Four patients' representatives participated including one as speaker.

- **Pharmacogenomics workshop - from science to clinical care**

The objectives of this workshop were to share international expert views, discuss challenges and identify areas for joint stakeholder action. Eight patients' representatives participated, including one as speaker.

- **Cystic Fibrosis workshop**

This two-day workshop focused on outcome measures for cystic fibrosis (CF) lung disease and exocrine pancreatic insufficiency, gathering experts in the field of CF together with regulators, pharmaceutical industry, and patients' representatives.

Two patients' representatives participated, including one as speaker.

- **Geriatric medicines workshop**

The fastest-growing segment of the total population is people aged 80 and over. To address this challenge, action in the geriatric field is a priority in the EMA Road Map 2015. The organisation of this

workshop is part of the EMA geriatric medicines strategy, involving stakeholders to discuss the initial steps and explore future directions.

Nine patients' representatives participated, including two as speakers.

- **EMA/FDA workshop on paediatric Gauchers – exploring the way forward**

This workshop was organised jointly by the EMA and the FDA, and included a specialised Gaucher patients' organisation who presented the views of families on recruitment into clinical trials, and the choice of ERT.

- **Workshop on development of new antibacterial medicines**

This workshop discussed aspects of the development of antibacterial medicinal products, including those targeting multiple-drug-resistant pathogens and with narrow-action spectra and discussion on specific indications.

Two patients' representatives participated.

- **Health related Quality of Life oncology workshop**

Organised by the EMA Oncology Working Party, this workshop the aim of this workshop was to gather information and opinions in order to generate a Health Related Quality of Life/Patient Reported Outcome (HRQoL/PRO) appendix to the general Guideline on the 'Evaluation of anticancer medicinal products in Man'.

Two patients' representatives participated; both gave presentations.

- **Joint EFGCP/DIA/EMA medicines for children conference on development of paediatric medicines: from learning to adapting.**

This conference discussed where we stand with paediatric drug development in Europe, where the partners in drug development agree and disagree, in which area the EU regulation can be considered a success, where the regulation might be modified in the future, where and how its daily interpretation by the EMA will be modified, and where collaboration between the involved players can be improved.

Four patients' representatives participated.

- **Joint TOPRA/EMA conference**

This annual conference was held in November 2012 and provided a review of the year as well as the outlook for 2013 and beyond. A representative from a patients' organisation was on the speaker panel.

Involvement in development of Eudravigilance

EudraVigilance is a web-based information system which provides data on suspected side-effects (adverse reactions) for medicines authorised in the EU. Since March 2012 EudraVigilance data is publicly accessible via the 'European database of suspected adverse drug reaction reports'; users can view the number of individual suspected side effect reports submitted for each centrally authorised medicine. (Reports for common drug substances used in nationally authorised medicines will be published during 2013).

A user-group, including eight patients' representatives has been in place since 2010 to assist in the implementation of the Eudravigilance access policy and have been very much involved in the preparation of a guidance document and on the preparation of the public interface. The group also

carried out usability testing on the site prior to its launch. The test website was also presented to the PCWP for discussion and feedback.

The group will continue to be involved in future phases and modifications of the website.

Input in other EU-wide initiatives

- **Enpr-EMA**

A PCWP member is part of the coordinating group of Enpr-EMA.

Each year a workshop is held; the fourth such workshop was held in March 2012.

Day one of the workshop was dedicated to strengthening the links and communication between all stakeholders: patient/parent organisations, network representatives, pharmaceutical industry and regulators. Day two was dedicated to discussions between members of the Enpr-EMA network; and also the Enpr-EMA coordinating group had their quarterly meeting to discuss and define priority tasks for the year 2012-2013.

Seven patients' representatives participated in the workshop.

- **ENCePP**

Patients' and consumers' representatives have participated in the meetings of the European Network of Centres for Pharmacoepidemiology and Pharmacovigilance (ENCePP) and one PCO representative is a member of the steering group since the beginning of 2010.

The aims of this initiative are to bring together the available expertise and research experience in the fields of pharmacovigilance and pharmacoepidemiology, to strengthen post-authorisation monitoring of medicinal products in the EU and to facilitate the conduct of post-authorisation safety studies.

- **PROTECT (IMI)**

Patients' representatives are involved in the PROTECT consortium (Pharmacoepidemiological Research on Outcomes of Therapeutics), funded by the Innovative Medicines Initiative Joint Undertaking (IMI). The EMA coordinates the project, managing a multi-national consortium of public and private partners.

This European project aims to strengthen monitoring of the benefit-risk of medicines by looking at the limitations of current methods and developing innovative approaches.

During 2013/2014, a significant increase in the involvement of patients and consumers in research activities surrounding the evaluation of benefit-risk methodologies is envisaged. Patients and consumers will be provided an opportunity to judge the relevance of the methods/tools for assessing and communicating the benefit and the risks of medicines.

Table 1. Activities involving patients and consumers at the EMA during 2012

Management board / scientific committees
MB (members)
COMP (members) and individual expert contribution
PDCO (members and alternates) and individual expert contribution
CAT (members and alternates, to be nominated in 2013)
PRAC (members and alternates, from July 2012)
CHMP ad-hoc consultations on medicinal products under evaluation
HMPC ad-hoc consultation
Working parties
PCWP (members, alternates and observers)
PhVWP (observers) up to June 2012
SAWP – participation as experts in the review of Protocol Assistance requests
Working groups
HCP WG (observers)
EudraCT Joint Operational Group (members)
Eudravigilance Users Group (members)
SAG / ad hoc expert group meetings
SAGs and ad-hoc expert group meetings – participation as patients' experts
Product information related activities
Review of package leaflets (new and renewal MA applications)
Review of new EPAR summaries
Review of safety communications
Other activities / meetings
Non-PCWP members attending PCWP meetings as observers
Training session for patients and consumers involved in EMA activities
Working group on funding and conflicts of interest of PCOs
Input on proposed symbol and text for package leaflet of medicines under additional monitoring
Meeting with thalidomide patients and victims organisations
Pharmacovigilance stakeholders forum meetings & prep TCs
Consultation on braille specifications
Surveys on EMA communication activities / documents
Review of revision of Q&A on biosimilar medicines
User-testing for EU clinical trials register website

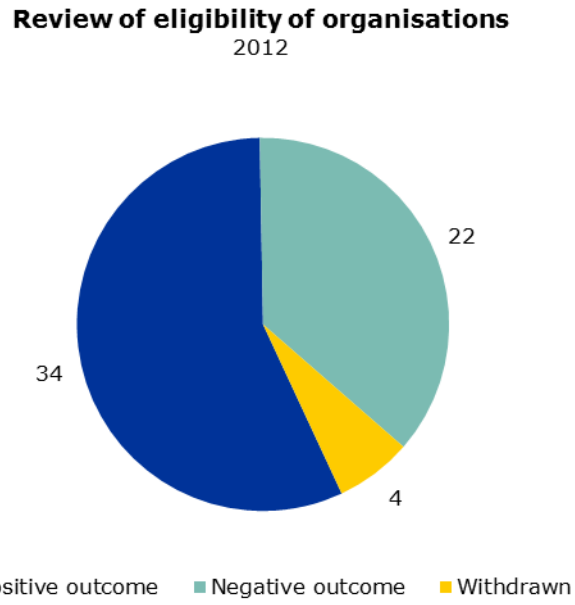
Workshops / conferences / info-days
Workshop on RMP summaries
EnprEMA workshop
Advanced Therapy workshop
Access to clinical trial data workshop
Pharmacogenomics workshop
Cystic fibrosis workshop
Geriatric medicines workshop
EMA/FDA workshop on paediatric Gauchers
Anti-bacterial workshop
Quality of Life Oncology workshop
EMA/TOPRA conference
Joint EFGCP/DIA/EMA medicines for children annual conference
ENCePP steering group meetings
Input on other EU-wide initiatives
European Network of Centres for Pharmacoepidemiology and Pharmacovigilance (ENCePP)
Pharmacoepidemiological Research on Outcomes of Therapeutics – (PROTECT – IMI)
European Network of Paediatric Research at the EMA (Enpr-EMA) – member of coordinating group

2. Organisations involved in EMA activities during 2012

By the end of 2012 there were 34 patients' and consumers' organisations included on the EMA list of "*eligible organisations"*.

Any organisation may apply to participate in the Agency's activities; however they must first become *eligible* by fulfilling the 'Criteria to be fulfilled by patients' and consumers' organisations involved in the European Medicines Agency activities'. These criteria are in place to ensure that the Agency works with organisations that are genuinely acting in the interests of European patients and consumers. All the eligible organisations are not-for-profit and their work is focused at a European level. Some are general umbrella organisations whilst others have a particular emphasis within a specific area (such as rare diseases, HIV/AIDS, cancer etc.).

A list of these eligible organisations is published on the Agency website, including links to their websites and a summary of their mission and objectives.



Occasionally the agency consults organisations not fulfilling all the criteria; due to the need to consult on a specific area, however this is in line with the "rules of involvement of members of patients' and consumers' organisations in Committees' related activities" (EMA/483439/2008 rev.1). They are listed in table 2b.

During 2012, a total of 57 patients' and consumers' organisations interacted with the Agency.

Table 2a: Eligible patients' and consumers' organisations working with the EMA

	NAME OF ORGANISATION
1	AGE Platform Europe (AGE)
2	Alzheimer Europe (AE)
3	Debra International
4	European AIDS Treatment Group (EATG)
5	European Cancer Patient Coalition (ECPC)
6	The European Consumers' Organisation (BEUC)
7	European Federation of Allergy and Airways Diseases Patients' Associations (EFA)
8	European Federation of Neurological Associations (EFNA)
9	European Gaucher Alliance (EGA)
10	European Genetic Alliances' Network (EGAN)
11	European Headache Alliance (EHA)
12	European Heart Network (EHN)
13	European Institute of Women's Health (EIWH)
14	European Liver Patient Association (ELPA)
15	European Multiple Sclerosis Platform (EMSP)
16	European Network of Fibromyalgia Associations (ENFA)
17	European Organisation for Rare Diseases (EURORDIS)
18	European Parkinson's Disease Association (EPDA)
19	European Patients' Forum (EPF)
20	European Public Health Alliance (EPHA)
21	European Prostate Cancer Coalition (EUomo)

	NAME OF ORGANISATION
22	Fabry International Network (FIN)
23	Global Alliance for Mental Illness Advocacy Networks (GAMIAN-Europe)
24	Health Action International (HAI)
25	Insulin Dependent Diabetes Trust (IDDT)
26	International Alliance of Patients' Organizations (IAPO)
27	International Bureau of Epilepsy (IBE)
28	The International Confederation of Childhood Cancer Parents Organisations (ICCCPO)
29	International Diabetes Federation European Region (IDF Europe)
30	International Patient Organisation for Primary Immunodeficiencies (IPOPI)
31	Myeloma Patients Europe (MPE)
32	Pain Alliance Europe (PAE)
33	Rett Syndrome Europe (RSE)
34	Thalassaemia International Federation (TIF)

Table 2b: Other organisations who interacted with the EMA during 2012 (e.g. participated in scientific advisory group meetings, scientific advice, workshops/conferences)

	NAME OF ORGANISATION
1	EuropaDonna
2	German heart foundation
3	Burns Unit Support Group (Chelsea & Westminster hospital)
4	Lymphoma association
5	National Ankylosing spondylitis Society (NASS)
6	PINNT
7	Heart UK
8	Myeloma U.K.
9	Fighting Blindness Ireland
10	Bettina Hausmann?
11	Parkinsons' UK
12	IBTA
13	Duchenne Parent project
14	Cystic Fibrosis Europe
15	Cystic Fibrosis Society UK
16	Aniridia Spain
17	Pro-Retina Deutschland
18	Spanish Federation of Retinitis Pigmentosa
19	Retina International
20	Batten Disease Family Association
21	Gaucher Association
22	UK MDS patient support group
23	Thalidomide associations: Thalidomide UK Agency, Thalidomide Society UK, FfdN (Scandinavian Society), Italian Association of Thalidomide Victims, Dysmelia, Spanish Thalidomide Association (AVITE), Dutch thalidomiders, The Irish Thalidomide Survivors Society

3. Number of patients' and consumers' involvement in EMA activities during 2012

During 2012, patients and consumers have been involved in 525 activities at the Agency (in several cases the same patient/consumer participated in more than one activity).

Activities have been split into three categories; 1) activities in which patients and consumers are members, alternates or observers, 2) activities involving individual experts, and 3) activities requiring organisations' representatives.

Table 3: Activities involving patients and consumers at the EMA during 2012

Membership of committees/MB	Members / alternates or observers
MB	2
COMP	3 / 1
PDCO	3 / 3
CAT	Pending nomination
PRAC (from July 2012)	Pending nomination
TOTAL	**12**

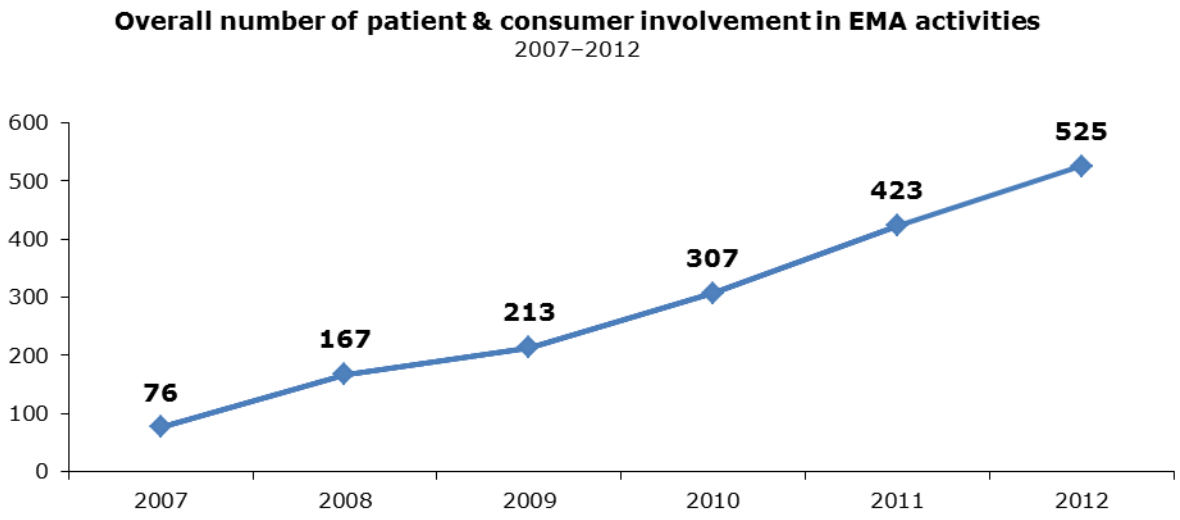
Membership of working parties	Members / alternates or observers
PCWP	15 / 13
PhVWP	1 / 1
HCP WG	2
TOTAL	**32**

Activities involving individual experts	Experts
Participation in Scientific Advisory Group (SAG)/ad-hoc meetings	36
Participation in SAWP meetings	19
Participation in COMP consultation	2
Review of safety communications (Q & A documents)	24
Review of EPAR summaries	36
Review of package leaflets	102
Participation in EMA annual training session	21
TOTAL	**240**

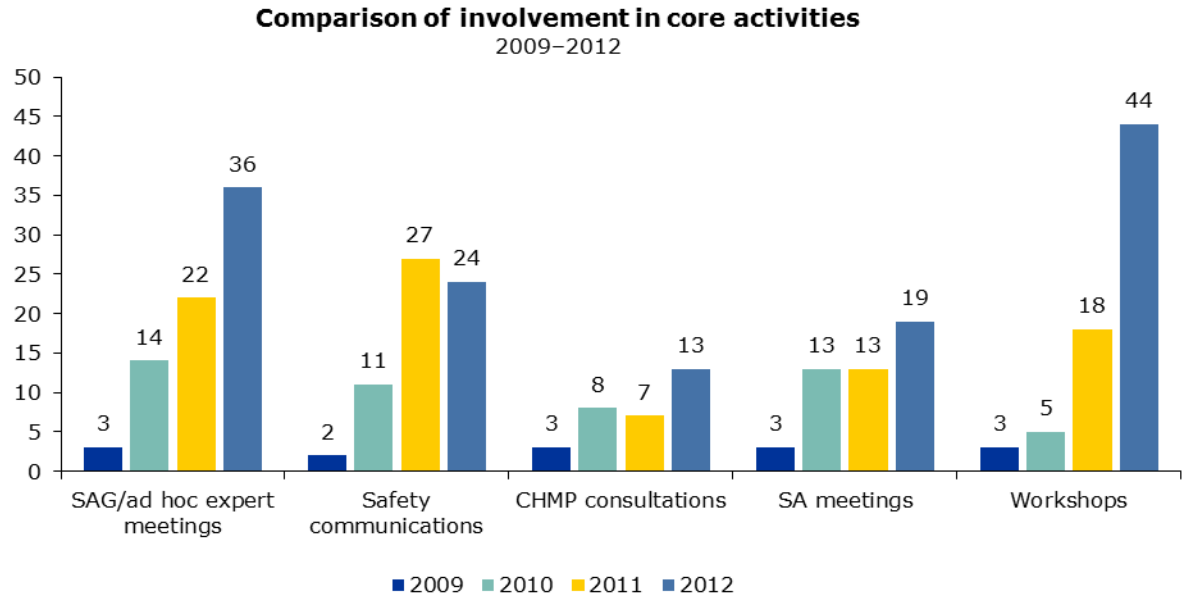
Activities involving organisation representation	Representatives
Ad-hoc observers/experts attending PCWP meetings	13
CHMP consultations on products under evaluation x 2	3
CHMP consultation with Thalidomide patients and victims organisations x 2	10
Patient organisation consultation with CHMP on a product under evaluation	1
PDCO consultation	2
HMPC consultation on PL advice on preparation of herbal teas	2
Consultation on symbol / text for medicines subject to additional monitoring	10
Presentation of patients' viewpoint on symbol & text to PRAC	1
Teleconference on EMA targeted information to patients and consumers	10
EMA survey on communication during pandemic flu crisis	13
EMA survey on communication documents	27
EMA consultation on braille specifications	4
Review of Q&A on Biosimilar medicines	4
Working group meeting on funding and conflicts of interest	11
Workshop on preparation and publication of RMP summaries	5
Briefing teleconference for stakeholders forum x 3	13
Pharmacovigilance stakeholders forum x 3	39
User testing for EU clinical Trials register website	5
EudraCT Joint Operational Group meetings x 4	22
Advanced Therapy workshop	4
Access to clinical trial data workshop	4
Pharmacogenomics workshop	8
Cystic Fibrosis workshop	2
Geriatric medicines workshop	9
EMA/FDA workshop on paediatric Gauchers	1
Anti-bacterial workshop	2
Enpr-EMA workshop	7
Quality of Life oncology workshop	2
Joint EFGCP/DIA/EMA medicines for children annual conference	4
EMA/TOPRA conference	1
Enpr-EMA coordination group meetings	1
ENCePP steering group meetings	1
TOTAL	**241**
TOTAL number of patients' and consumers' involvement during 2012	**525**

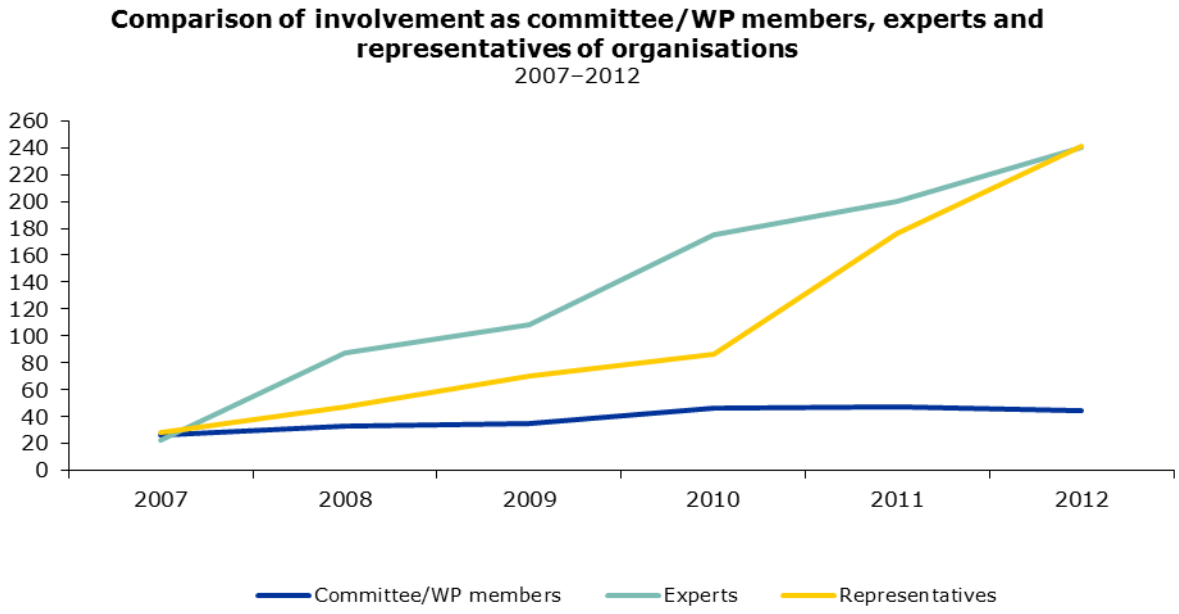
3.1. Involvement of patients and consumers in EMA activities: comparative analysis of data from previous years.

The graphs below provide details of the patients and consumers who have been involved in the Agency's activities and compares them with previous years.



The figures show that, compared to previous years, the involvement of patients and consumers in the different activities of the Agency has again increased during 2012.





- **Members:**

The number of members, alternates and observers part of EMA committees and working parties has remained more or less constant, as would be expected.

- **Experts:**

240 experts were involved in Agency activities during 2012. There has been an increased involvement in:

- SAG/ad-hoc expert meetings
- Scientific advice meetings
- Review of package leaflets

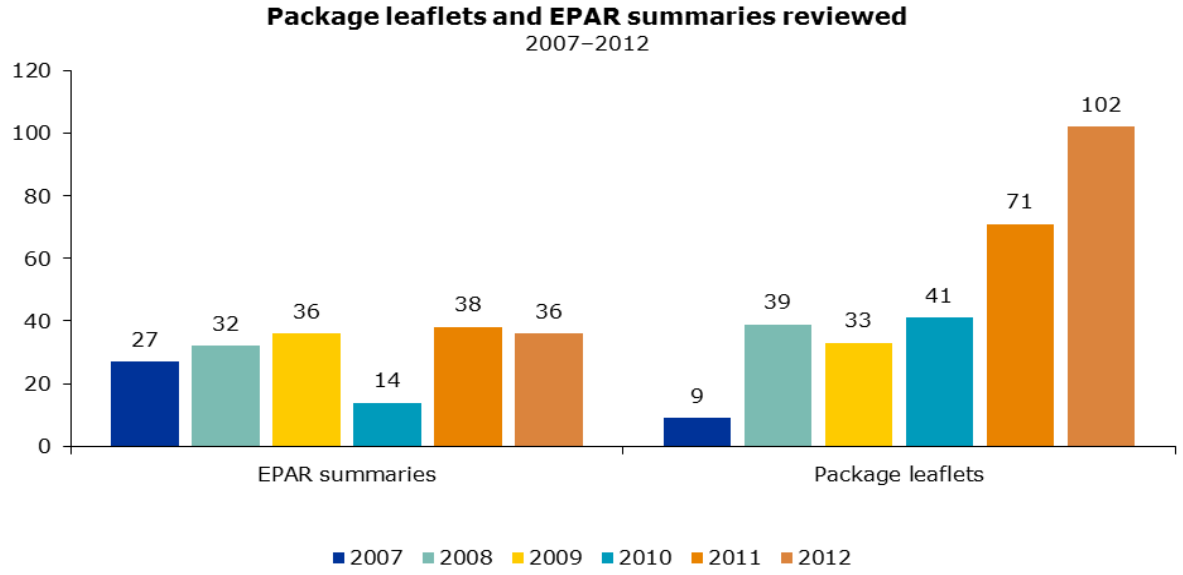
- **Representatives:**

57 different organisations interacted with the Agency during 2012.

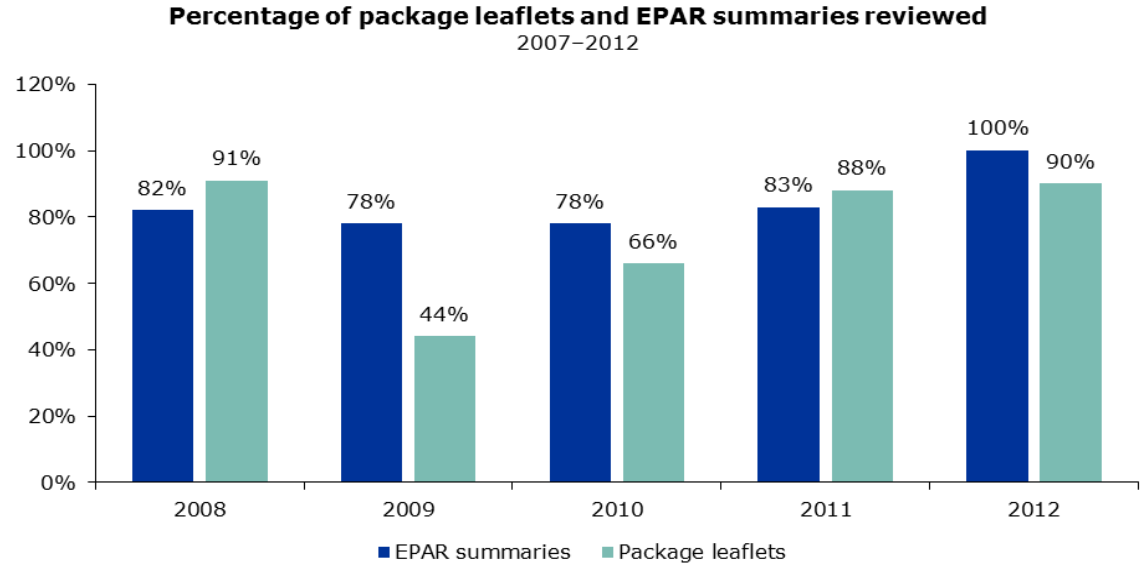
The increase in involvement of patients' and consumers' organisations' representatives can be attributed to committee consultations, EMA consultations and pharmacovigilance legislation stakeholder forums, as well as increased participation in EMA workshops, conferences and working groups.

3.2. Document review procedure: comparative analysis between 2007 and 2012

The graph below shows the number of EPAR summaries and package leaflets sent for review. There has been an increase in the number reviewed; especially package leaflets (mainly renewals).



The graph below shows the percentage of EPAR summaries and package leaflets reviewed (in relation to the number sent out), which demonstrates a very high response rate.



4. Conclusions

During 2012 the involvement of patients and consumers in EMA activities has again increased. This growth relates mainly to an increased participation in SAG and ad-hoc expert group meetings and scientific advice procedures, an increased review of package leaflets, activities related to the implementation of new pharmacovigilance legislation, and increased participation in workshops and conferences, in addition to the usual continuing activities as described herein. It is not expected that the numbers of patients and consumers involved will continue to increase in this way in the future; it is likely that a plateau will be reached in the next year or so.

This collaborative interaction allows patients to share their experiences with the EMA on issues that affect them and in doing so, they provide meaningful feedback on the real-life implications of regulatory outcomes, which ultimately contributes to the quality of the medicines assessment and outcome. This is stressed within the Agency's roadmap to 2015 which emphasises that the decision-making process can be improved by taking account of patients and consumers experience.

Patients and consumers are now a familiar part of the Agency's work and with the passing years their involvement has not only expanded but has evolved and been refined, always endeavouring to achieve optimal and appropriate involvement. One example is the growing participation in benefit/risk deliberations; where patients inform what benefits are meaningful and what risks are acceptable to them and whether, in their point of view, a particular balance of benefits and risk is favourable or not. June Raine, the PRAC chair, confirmed that "the patients' contribution to a robust benefit-risk evaluation is vital".

Francois Houyez, representing EURORDIS and involved with the Agency since 1996, confirmed the true collaboration which has developed between the EMA and the patients' groups: *"The Agency listens and takes into account the views, opinions and information provided by the patients. Consideration of our views is not purely formal, it is real, constant, and can be measured. There has been a strong will to involve the patient in the regulatory process since the creation of the Agency, and this will has developed since then. It puts our contribution at the same level as those of other experts, no more, no less. Eventually, it benefits the evaluation process, in the interests of the patients."* (I. Moulon, Global Forum;" Interaction between the EMA and HIV Advocates/patients over the years")

Next steps

- It is intended that the revision of the "Framework of interaction" between the EMA and PCOs will be finalised during 2013, including:
  - A definition of the specific role of patients and consumers within the different scientific committees of the Agency, which has already been finalised & published.
  - A training strategy which defines in detail the specific training that is provided to patients and consumers, depending on the activity in which they are involved, which has been finalised and will soon be published).
  - A definition of how patients and consumers are involved in the benefit/risk assessment of medicinal products, highlighting criteria and format of involvement; a workshop will be held during 2013, during which a dedicated document/paper is expected to be finalised. This paper will outline the way patients and consumers can be involved within the benefit/risk deliberations related to the evaluation of medicines.

- PCOs will continue to be involved across the Agency, providing input within many EMA activities, as well as being invited to take on new tasks that may arise, especially related to the current discussions on increased and/or refined involvement in benefit/risk discussions.
- Patients will become involved in the scientific advice procedures for all medicines (not just those for rare diseases); highlighting aspects of their daily lives that can then be incorporated within the clinical trials.
- The EMA, together with the PCWP, will finalise its endeavours to improve the way potential conflicts of interest of organisations related to their funding are handled.
- The training and support provided to patients and consumers, tailored to specific activities, and detailed within the training strategy, will be fully implemented, ensuring clarity on scope of specific roles, tailored induction, support and briefing, as well as continued monitoring and feedback and proposals for improvement where needed.
- The Agency will continue to look to increase the network of eligible organisations in order to cover as many therapeutic areas as possible and will also highlight to the organisations that there are many opportunities for lay people, and the organisations that represent them, to be involved with EMA's work.
- The next update report will be presented to the PCWP and the Management Board in 2014.

SECTION 2:
Analyses of the degree of satisfaction of patients and consumers involved in EMA activities during 2012

Since 2007 the Agency has been measuring the degree of satisfaction of patients and consumers who have been involved in EMA activities, as requested by the EMA management board. This is an essential tool to monitor the interaction and to identify potential areas for improvement and future actions.

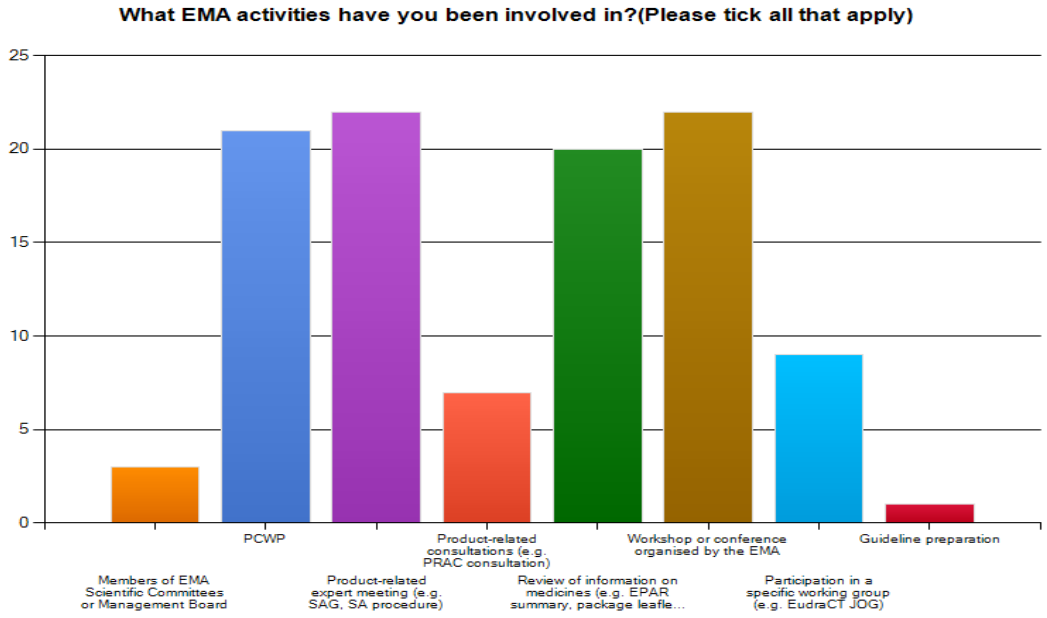
Every patient and consumer involved in EMA activities during 2012 was sent a questionnaire (approx. 160 patients and consumers) and 53 responses received (33%). It could be completed anonymously.

The questionnaire consists of 15 questions, which could be answered by choosing among 5 grades of satisfaction rating from “Very satisfied”, to “Very dissatisfied” and each question also provided an additional box where the respondent was invited to add additional comments.

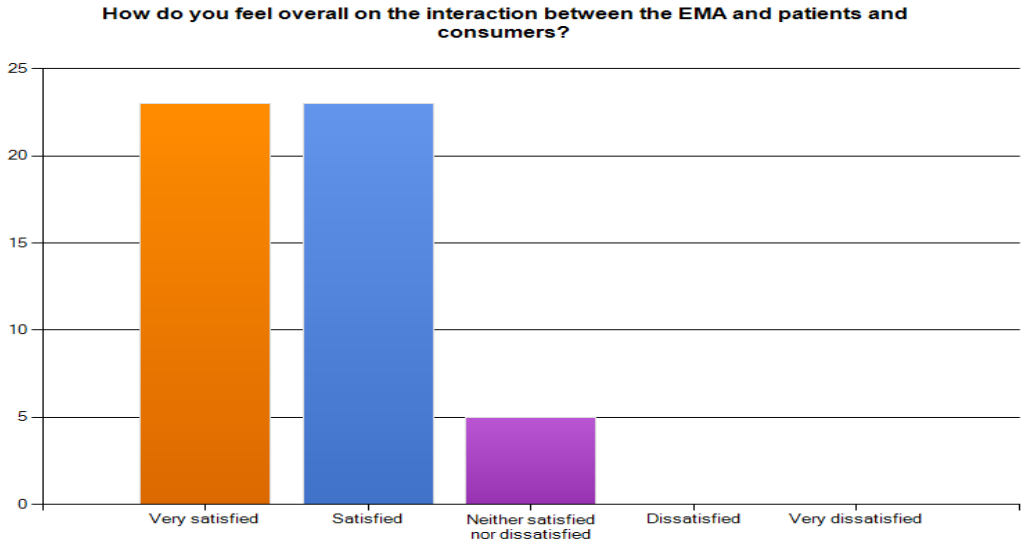
There are 4 main sections, Activities, review of documents, general interaction, and logistics, including financial support.

The results were collected and analysed and are shown in the graphs below.

Activities

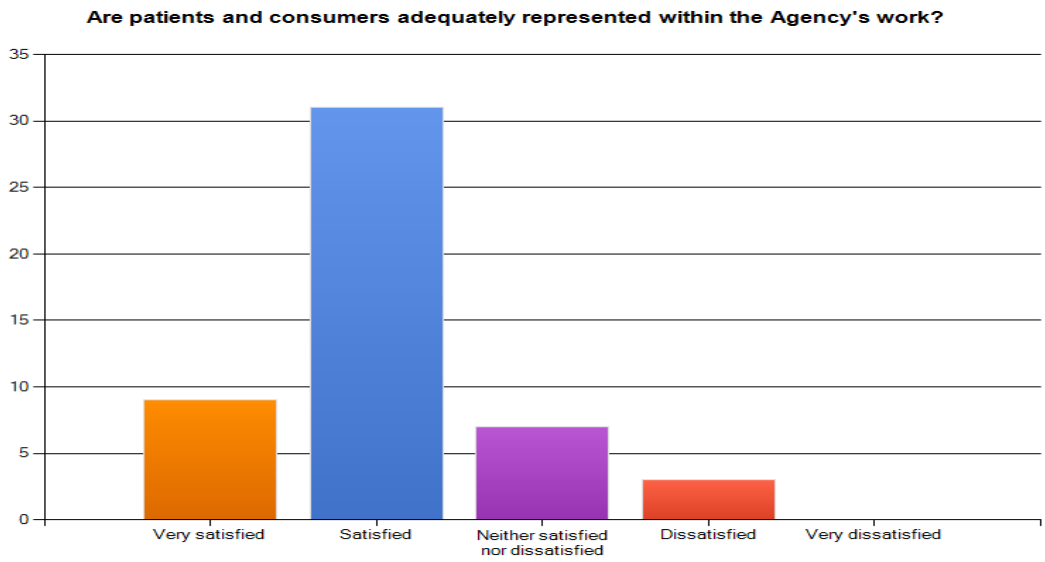


General interaction



The majority of responders indicated that they were satisfied with the overall interaction, with a significant number being *very satisfied* and none being *dissatisfied*. The level of satisfaction has increased compared to the 2010 questionnaire results.

Some comments mentioned that "*Compared with other agencies, EMA is setting a very good example*", and "*I always use the EMA as the best example of meaningful involvement of patients; however the CHMP should accept patients too*". However it was also mentioned that "*It is quite good but it feels as if the consumers and patients cannot really change things due to the working of the agency*", "*Patients and their representatives should be invited to comment more often to specific questions*" and "*EMA could be much more pro-active towards the outer world*".

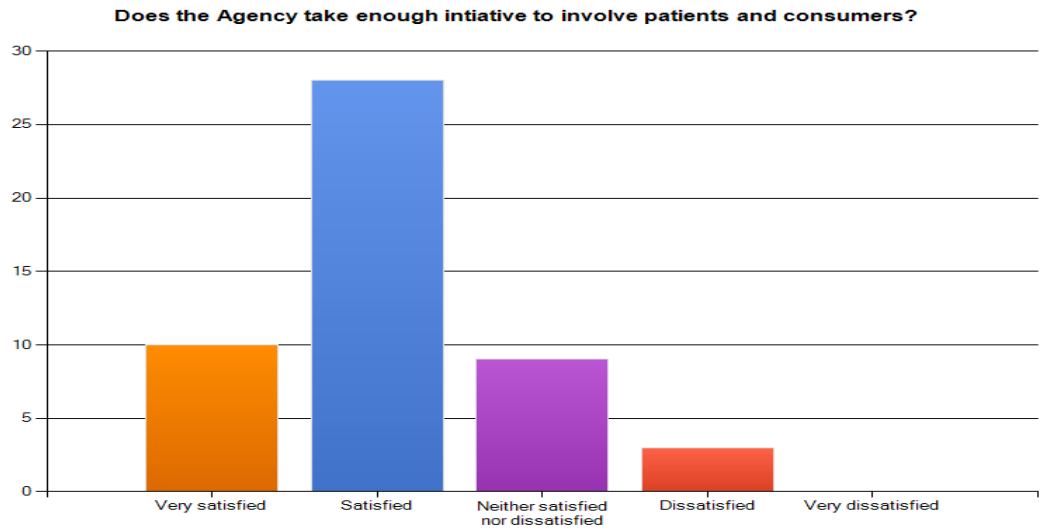


Most patients indicated that they were satisfied that patients and consumers were adequately represented within the Agency's work and again an increase in overall satisfaction compared to the previous questionnaire. Some individual comments received were: "*Unfortunately it always appears to be the same people*" and it "*depends on the subject*", and "*This is increasing all the time and emphasis is being placed on this aspect*"

It was also mentioned that "*1 patient member at the PRAC in not enough and there are still committees without a patient representative*", similarly that "*CHMP is missing*" and "*There is still room for improvement in the future, for example considering the HMPC, but feel the agency's commitment to the principle of involving patients and consumers is commendable*".

Areas for improvement have been suggested as follows:

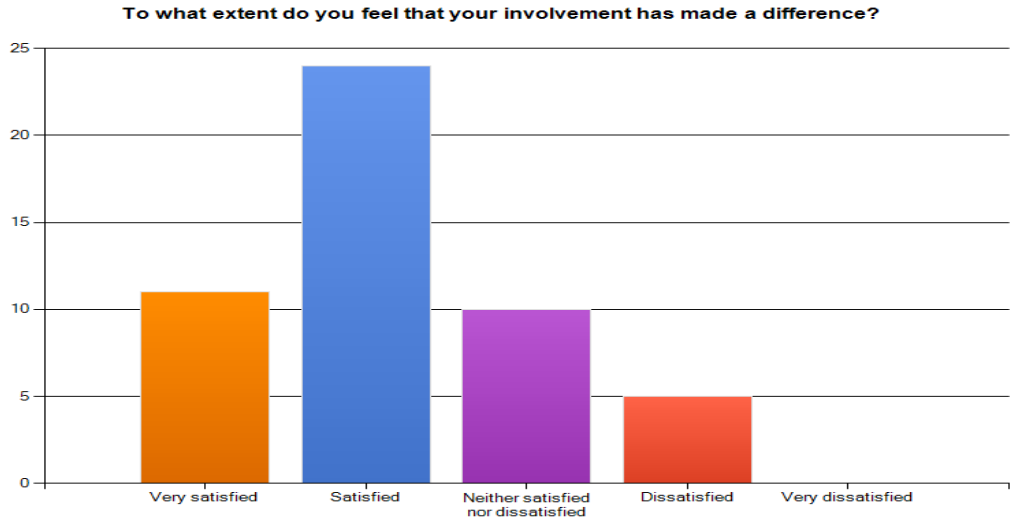
- Further involvement in benefit-risk evaluations (revision of framework).
- Systematic involvement in cases such a product shortages.
- Increasing the network of patients involved within the Agency and look to expand the eligible organisations.



Most responders were satisfied with the way that the Agency takes initiative to involve PCOs in its work (similar results to 2010 questionnaire). Some felt that "*sometimes it feels like that, other times not*" and that "*it can be done more*". Overall it seems that "*this is an area that requires time and personnel and initiative is definitely growing and emphasised*"

Areas for improvement have been suggested as follows:

- Continue to provide financial support for PCOs coming to the Agency (where appropriate).
- The Agency to continue looking at additional areas in which PCOs involvement can be enhanced in terms of benefit/risk evaluations.



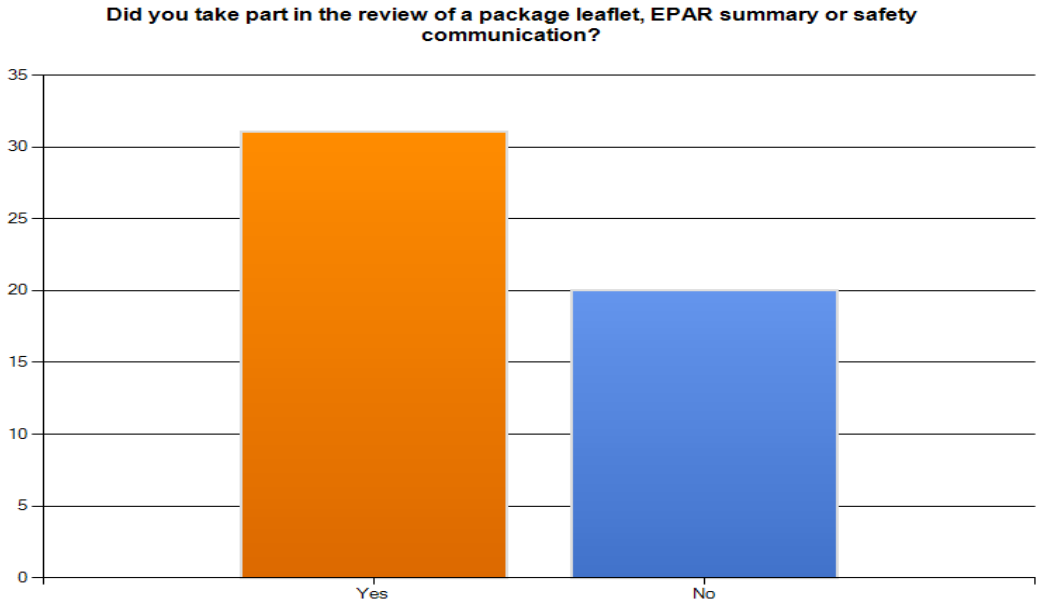
Many responders were satisfied that their involvement had made a difference, although a number responded neither satisfied or dissatisfied, and mentioned that "*I have been to only a few meetings and feel my contribution is still insufficient*" and "*I can't really say that, had the feeling that I could express my opinion, but not sure if it really made a change*". The results showed a slightly higher *very satisfied* rate compared to 2010 but also slightly higher *dissatisfied*. This could also be attributed to the fact that it is not always evident to patients whether or not they have had an impact, and if so, how much.

Most patients were happy with the level of training and support they received, although several mentioned they "*did not have any training*" and "*would have wished a bit more*"

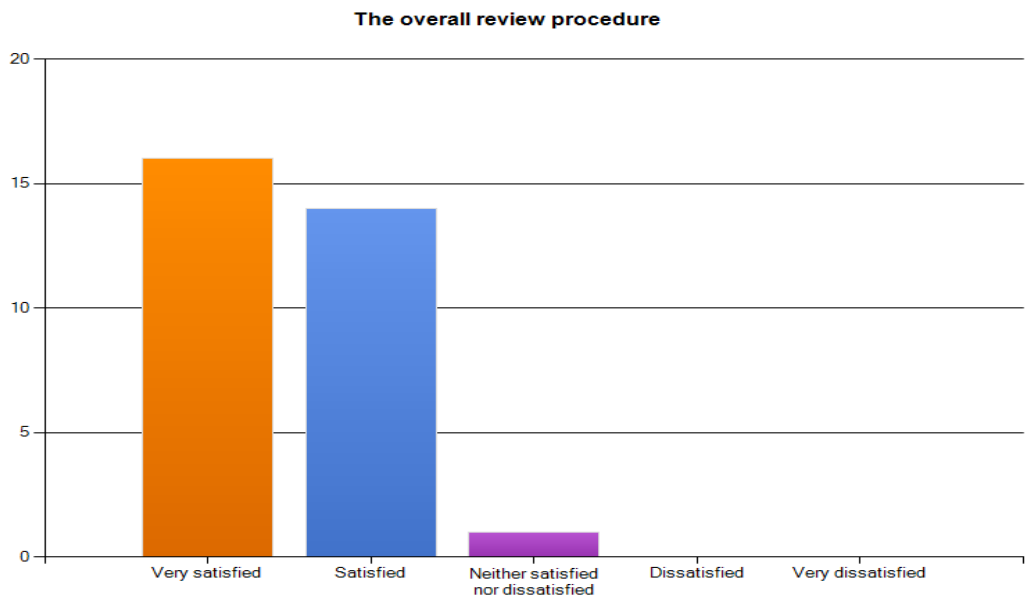
Areas for improvement have been suggested as follows:

- Ensure that the strategy on training and support adopted in 2013 is fully implemented and to look at ways to increase and/or improve the current training material on offer.
- Training will be evaluated within future performance indicators and measures taken if necessary.

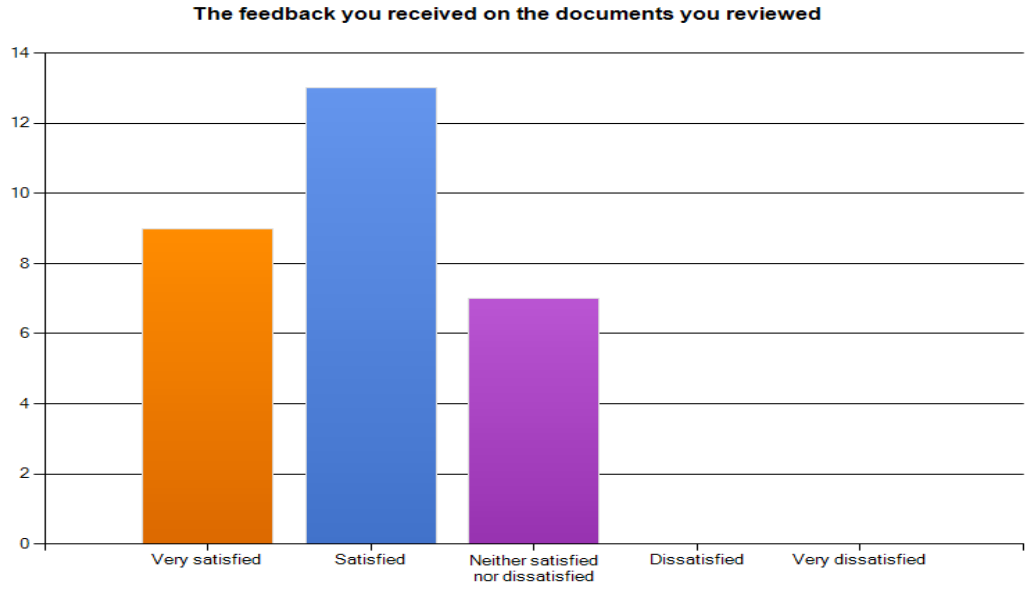
Review of documents



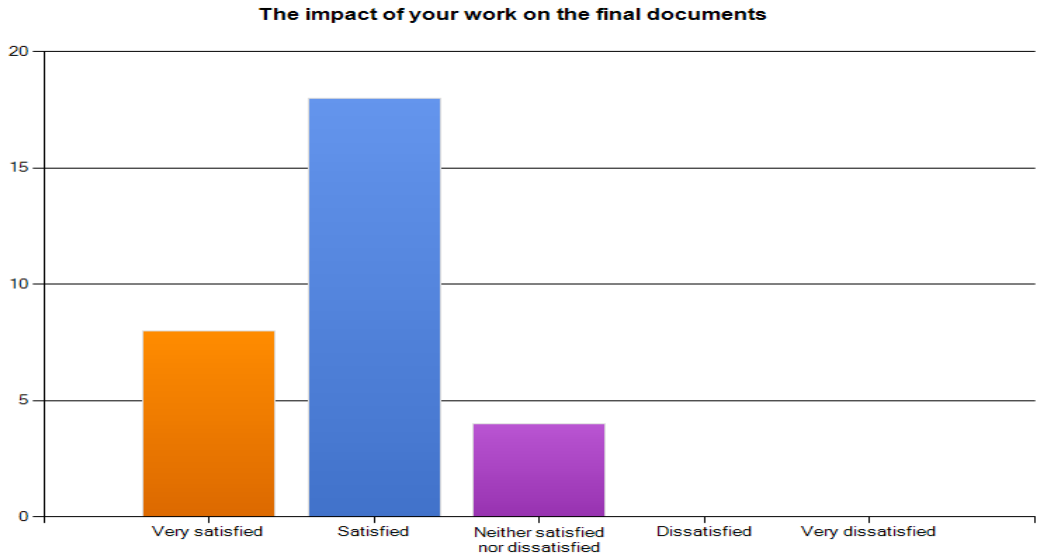
If, yes.



The majority of responders were either very satisfied or satisfied with the overall review procedure. It was mentioned that "*Sometimes the deadlines are very short and it can be a challenge to reply in time*." The numbers responding 'very satisfied' is significantly higher than in the previous questionnaire.



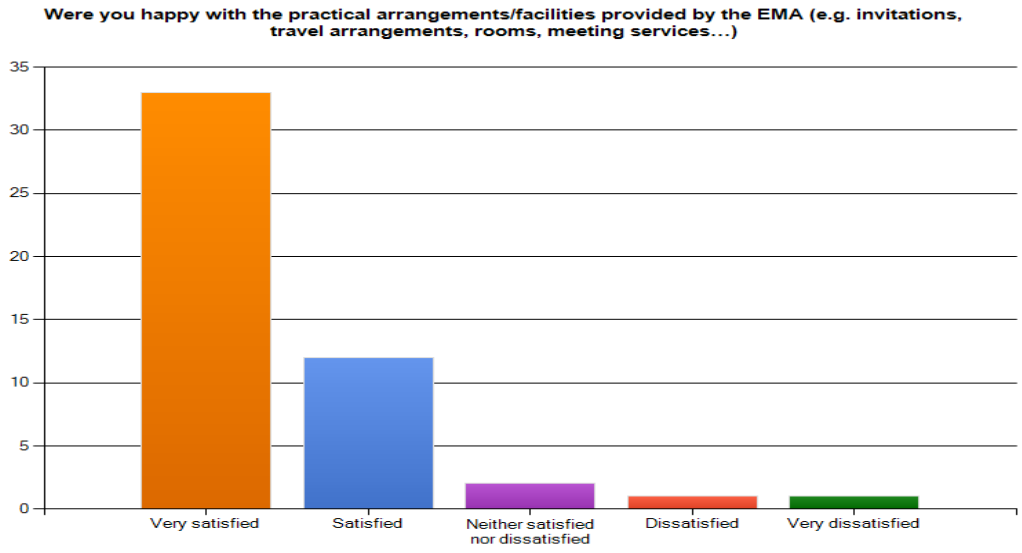
Most responders were either very satisfied or satisfied with the feedback they received, there were a number of neutral responses, likely due to the fact that they did not receive individual feedback.



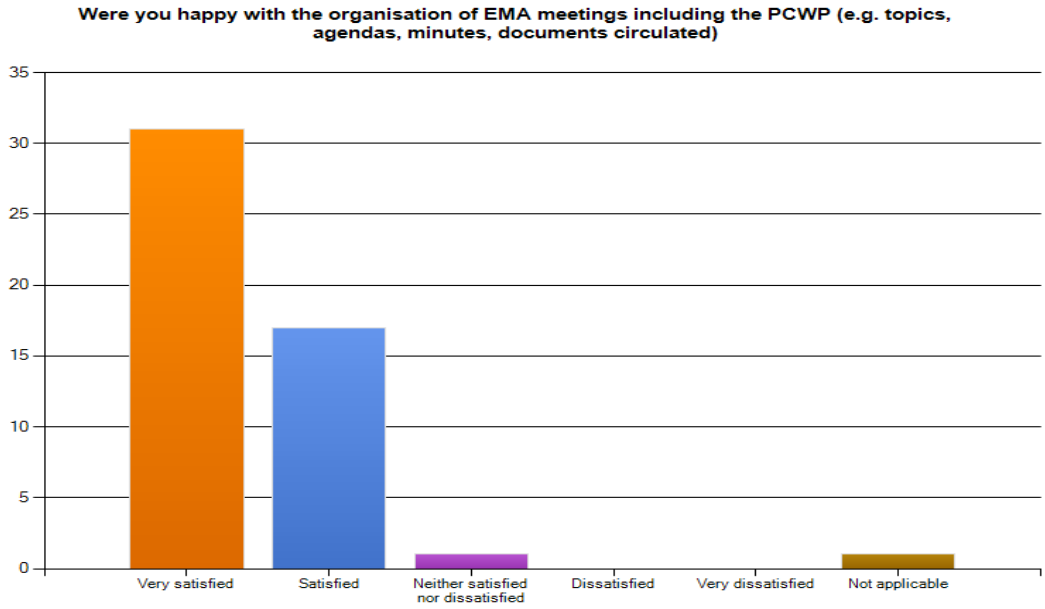
Again most responders were satisfied with the impact of their work, with a number of responses being neither satisfied nor dissatisfied, mentioning "*Do not really know*" and "*Did not receive any comments on leaflet revisions*"

Note: When patients' representatives review package leaflets, they are sent the version with all accepted comments (after discussion at the QRD review group) enabling them to see if and how their comments have been incorporated. Those reviewing EPAR summaries are sent individual specific feedback on their comments.

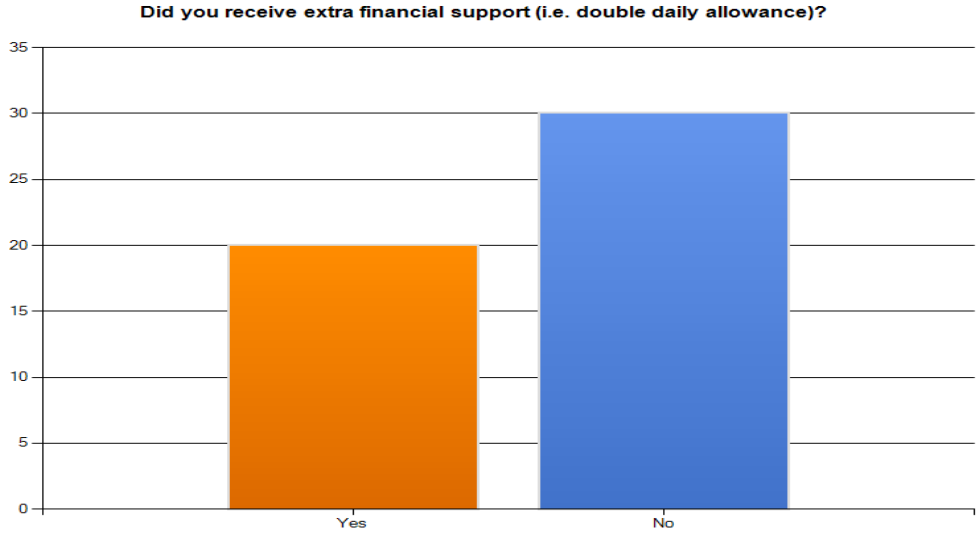
Logistics



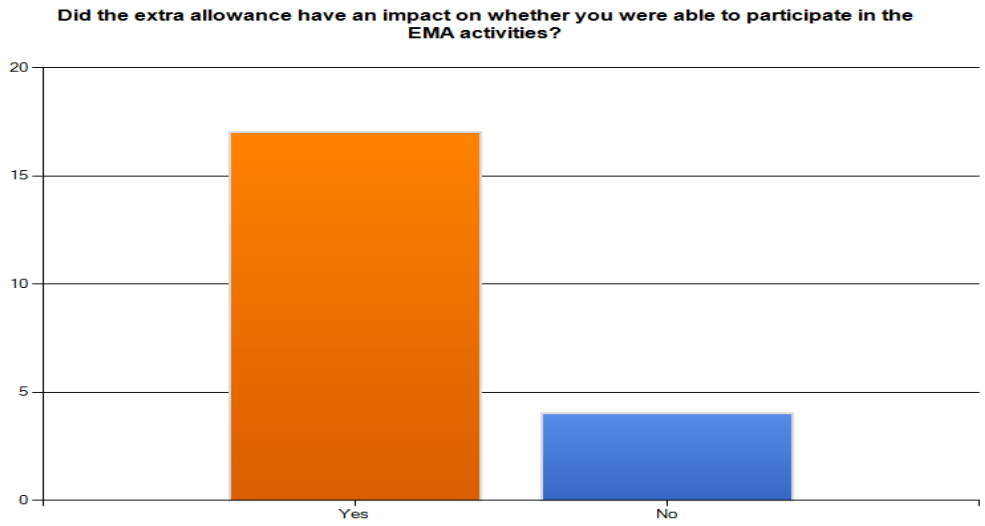
Overall most patients were very satisfied with the practical arrangements at the EMA (improved since 2010), although it was highlighted that the "*reimbursement policy is too complicated*" and that there is "*a lot of bureaucracy in the beginning, but then excellent*"



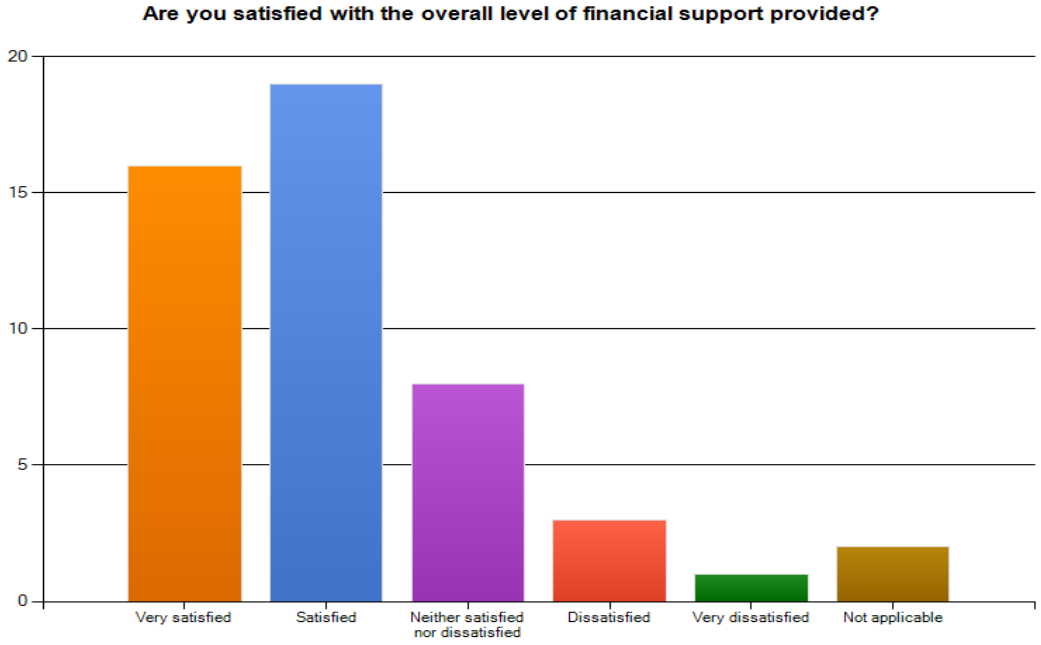
Again most patients were very satisfied with the organisation of meetings at the EMA, mentioning that information was "*Timely received*".



A higher number of patients (who responded) received the extra allowance, compared to 2010.



Many PCOs felt that receiving extra financial assistance did have an impact on their participation in EMA activities. Some comments included: "*Without such an allowance I could not participate*", "*as volunteer the extra allowance is highly appreciated*" and "*This makes a lot of difference*". However it was also said "*allowance level not sustainable*" and "*It would have to be substantially higher*".



The majority of patients were satisfied with the level of financial support provided. However it is clear that for some participants it is not sufficient. For example: "*since the introduction of telephone conference calls, one can be on the phone for hours, yet no compensation is paid for this*". "*In groups like PhVWP there is a lot of workload, so more financial support should be provided*", "*I am aware we should not attend for the money, so in that regard the allowance is good. However, I am working as an independent and am unable to work more than 3-4 days/week for health reasons. The fee is substantially lower than my charge-out rate and means I need to turn down paid work*."

Areas for improvement have been suggested as follows:

- Explore support for patients working on EMA activities in addition to participation in meetings.

Overall conclusions on 2012 performance indicators questionnaire

The results and analyses of the performance indicators questionnaire demonstrate that overall general satisfaction remains high from PCOs who have been involved in EMA activities during 2012. This is also emphasised within the general comments included, especially at the end of the questionnaire.

The level of satisfaction has increased in several areas compared to the results of the questionnaire completed by PCOs in 2010.

Additional financial support included since 2009 is appreciated and perceived as an EMA understanding of the value of patients' contributions, however it is felt that this could be increased and also extended to include time spent on activities outside of meetings (e.g. experts involved in the review procedure and written consultations).

The involvement of patients and consumers continues to be a mutual success, from both PCOs and EMA perspectives and the increased levels of satisfaction demonstrate that measures put in place have been effective.

General comments from PCOs

At the end of the questionnaire there was an area for general comments on the overall interaction between PCOs and the Agency. Some of these comments are included below:

"I feel that the EMA have been very welcoming to patient and consumer organisations. More patients are becoming more involved in the work of the EMA. The Conflict of Interest policy has caused a lot of discussion and is still not finalised to everyone's satisfaction. I have really enjoyed being involved in the EMA activities. I feel we as patients representatives are consulted, listened to and our advice has helped shape the EMA policy and outcomes."

"EMA might think about remuneration of volunteers who only make written contributions".

"I hope for: - more involvement of consumers in the EMA activities - a simplified language for the EMA website, especially in the area of EudraVigilance and more transparency in each EMA activity".

"I had a very interesting experience. I was welcomed and invited to comment during the meeting. The event was well planned and I was given all the information I needed by phone and email in advance".

"Throughout the meeting I was recognized by the attending Medical Experts as a community member with sound understanding of the debated subject matter. This recognition made me feel valid and suitable for the meeting."

"The papers supporting the meetings are written within a high scientific standard, which is complicating mostly simple facts. I guess that all things could be expressed in a much shorter and simpler form. The actual form of documents requires a lot of paper as well as a lot of pages during the presentation by power point. A general problem seems to be that a lot of patients throughout Europe cannot participate in EMA activities, because they are not trained in conference English language".

"I think EMA should concentrate on the difficulties patient organisations have with the local regulatory agencies that very often do not listen, invite, and involve local patient association. In other words, your best practices are not taken by local agencies and this is a pity. Another point not yet resolved, is the conflict of interest policy."

"I was very happy that I got the opportunity to attend a SAG meeting and felt our contribution was really sought after".

"My 5 years of experience with EMA has been very rewarding. When I compare to the efforts put in by other Agencies with respect to patients, EMA sets a very good example. If the issue here is financial support, our position on this is that patients' representatives who are volunteers for their own associations and take a significant amount of time to be involved in EMA activities certainly suffer from loss of salary during that time as well as additional travel, reading, conference call and follow up burden with no compensation. I very much appreciate the efforts that have been put into promoting EMA activities such as the COMP role including vice Chair and the excellent SAG video. It would be great to see more of this type of tool. One final comment is on the consistent helpfulness and accessibility of the staff at the Agency from all levels. This is very much appreciated and makes the Agency a very nice place to come to and more importantly to send patient representatives to".

"I would like very much to be more involved in provision of patient information, by creating adjusted text for the wider audience who have less knowledge of the used professional nomenclature in order to achieve a better involvement of the wide population in the UK. It is my awful experience that amongst the population, the EMA is a totally unknown institution, let alone what it does or could do for them".

"I am satisfied with the overall level of financial support since it covers my expenses. It might be different for other members".

"In my view the EMA gives good support for me/my organisation's participation. However, from our side the barriers to more intensive or possibly more effective participation are due to a simple lack of human resources. Liaison with the Agency is often around quite technical topics and requires quite a lot of background work as well as time. Ideally we would have one person in the secretariat who could concentrate on issues around pharmaceuticals regulation and legislation, but this is currently not the case. I can imagine this being even more of an issue for smaller organisations who rely on really minimal staff/volunteers".

"Sometimes we think that it is important to have translators, some things are very important and we must understand them perfectly. But it's really fantastic".

"It would be great if, in the future, presentations could be written either in bold or in slightly bigger letters. Sometimes, it is difficult to follow on the screens".

"The staff is very nice and helpful!"

"I am very pleased with EMA - Very good organization – Useful information".