

European Medicines Agency's interaction with patients, consumers, healthcare professionals and their organisations

Annual report 2016



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We would like to acknowledge the leadership and commitment of Dr. Isabelle Moulon who has championed the involvement of patients, consumers, healthcare professionals and more recently academics and researchers in the work of the European Medicines Agency for more than 20 years. As co-chair of the Patients' and Consumers' Working Party (PCWP) and the Healthcare Professional's Working Party (HCPWP) until 2017, she has played a key role in successfully making the stakeholder voice an integral part of the regulation of medicines at the European level. We are pleased to continue collaborating with her as EMA senior advisor to stakeholders and we would like dedicate this Annual Report to her.

About the annual report

Reporting the European Medicines Agency's interactions with stakeholders is a key element of the frameworks of interaction with patients and consumers and with healthcare professionals.

Starting in 2007 as a report covering the 'progress of interactions' with patients and consumers the document developed into a detailed account of activities where these stakeholders had been involved over the year. With the adoption of the framework of interaction with healthcare professionals, the report was then expanded in 2013 to incorporate interactions with all these stakeholders. Today, the report reflects the refinement of the scope of interactions, emphasising not only the interactions with individual patients, consumers and healthcare professionals but also with their representative organisations.

As we progress further with structuring collaboration with academia, the report will continue to adapt to reflect the evolving change and acceptance of the integration and importance of the work and input of these stakeholders in the regulation of medicines and associated activities.

The EMA would like to thank the members of its eligible organisations and all the individual experts who generously contribute their time and experience to ensure the continued high quality of all aspects related to medicines in the European Union.

Executive summary

2016 was the occasion of the 10th anniversary of the Patients' and Consumers' Working Party (PCWP). This platform evolved from the working group with patients and consumers and continues to be an integral point of contact with patients and consumers and represents an important mechanism for EMA to interact with these stakeholders. This unique group has been central to many decisions and along with the eligible organisations represent the range of diseases covered by the mandatory scope of the Agency and beyond.

2016 also saw the beginning of the new mandates for both working parties, which will span the period 2016-2019. Elections for the working party co-chairs were also held; Gonzalo Calvo was renewed by the HCPWP for another three years and the PCWP elected Kaisa Immonen as their co-chair.

Concurrently, the work plans for the working parties have been structured to address not only the annual work plans but also a longer term vision taking into consideration the EU medicines agencies network strategy to 2020 and the EMA multiannual work plan.

The year was also marked by the revision of the framework of interaction of EMA with healthcare professionals, first adopted in 2011. The revised framework builds upon the original document and incorporates the experience gained thus far by recognising that advances in adaptive design for clinical trials, personalised medicine, more extended use of real world evidence, and the public availability of clinical data will provide additional opportunities for engaging with healthcare professionals in the interface of clinical research and clinical practice.

Another objective for 2016 was to engage more with general practitioners and in this context a workshop was organised. The importance of the perspective of these groups on public health and the impact of regulatory decisions is clear and the Agency is liaising to determine how best involve them in its work.

A further framework has been created, which is to describe collaboration with academics and researchers. Although it may be difficult to separate clinical practice from research and education, the healthcare professionals' framework mainly focuses on clinical practice whilst the academia framework will put its emphasis on research and education, without pre-empting obvious areas of inter-relation. Finally, and very importantly, the frameworks should complement the established collaboration with patient and consumer organisations.

One goal of the revised framework for patients and consumers was to establish a pool of individual patient experts to ensure participation throughout the lifecycle of medicines. These individual patient experts would not necessarily be affiliated with any particular organisation. While the eligible organisations remain the first port of call in the identification of patients for EMA activities, the expansion and increase in activities where patients are involved has required additional resources. A call for expression of interest was launched for the individual patient database was launched in January along with supporting information about new database. Patients have already been registered and involved at EMA via this new tool.

The pilot to include patients in plenary meetings of the CHMP was concluded. Patients were invited to provide input during six oral explanations on specific medicines. A report of the experience and outcome was presented to CHMP and will be published in the first quarter of 2017. This report includes the final results of surveys to CHMP members and patients who attended.

Patients have also been involved in the review of the summary of herbal medicines since March and 29 patients reviewed 25 of these summaries in 2016. The group of reviewers will also be invited to attend HMPC meetings as observers during 2017 to meet committee members and see first-hand how the

committee works. As with other committees, patients will also then take part in ad hoc consultations by the Herbal Medicinal Product Committee (HMPC), as needed.

Several of the working parties topic groups, whose members include patients, consumers, healthcare professionals and EMA, concluded in 2016 having made their recommendations and have begun the process of implementation, where possible. These recommendations will be presented in a report along with an update of the current status of the other groups. One ongoing topic group is that for the involvement of young people in Agency activities, where guidelines are currently being approved by EMA management to enable the involvement of young people in EMA activities. Another ongoing topic group will see its scope enlarged in 2017 from social media to digital media and health, in order to also encompass developments in mHealth and real-world evidence. The topic groups have been a very helpful way to engage with all EMA eligible organisations in the margin of the working party meetings in order to exchange and discuss on issues of common interest.

Future steps

As with previous years, it remains of utmost importance to continue to provide support for all activities where patients, consumers and healthcare professionals are invited to participate in EMA activities. As these activities develop and diversify, we will see greater emphasis on coordination, preparation and communication of outcomes in order to sustain meaningful, timely and high quality involvement of stakeholders in EMA's work.

Whilst keeping a broad overview of different areas of interest in clinical practice and clinical research, EMA will continue to reinforce and promote engagement with general practitioners, in close collaboration with their representative European organisations. The EMA will also look at additional methodologies for gathering patient input, specifically from larger groups of patients, when needed. Following on from a small pilot in 2015, the Agency will continue to explore the use of survey methods for obtaining patient input for EMA assessment.

With all these activities there is the need to simplify and improve interactions with stakeholders and put in place practices to reduce the shared burden associated with involvement in regulatory activities. Further supporting their participation would include making better use of existing training tools and materials and developing new ones

The EMA is moving closer to public engagement – via the further promotion and use of the individual database and implementation of public hearings, the first of which is likely to take place in the second half of 2017.

In accordance with work programme to 2020 of EMA and the common strategy of the European regulatory network, Personalised Medicine and antimicrobial resistance will be highlighted and workshops will be organised for information and training in 2017

This report was circulated to the joint PCWP/HCPWP and was presented to the Management Board during its meeting on 15 June, 2017.

1. Areas of common interest and collaboration for patients, consumers and healthcare professionals

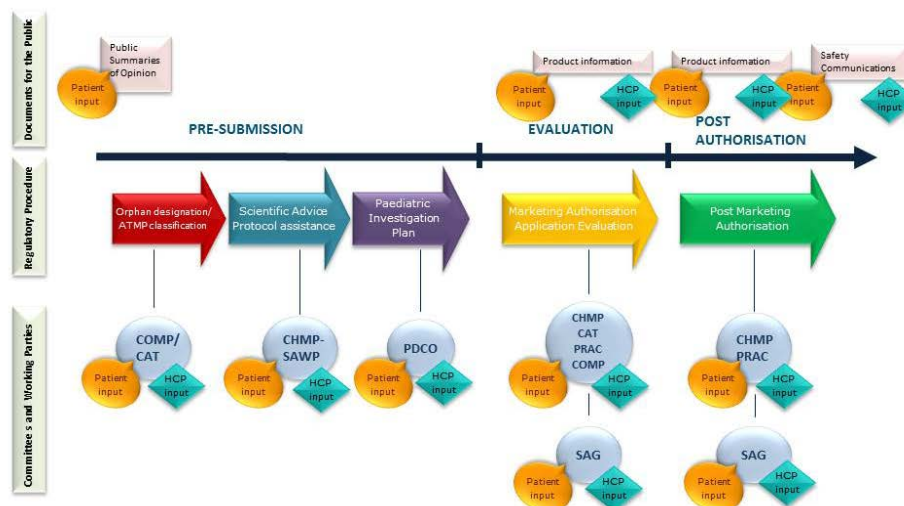
1.1. Introduction

The Annual Report of EMA's interaction with patients, consumers, healthcare professionals and their organisations provides a comprehensive description of the activities of these groups in the work of the Agency.

The last two decades have paved the way for full integration of patients, consumers and healthcare professionals all along the regulatory lifecycle of medicines at the Agency (Figure 1: Regulatory lifecycle of medicines and involvement of patients/consumers (orange bubbles) and healthcare professionals (green diamonds) Figure 1).

The 'Patients and Healthcare professionals' department became the 'Public Engagement' department to better reflect the expanding group of stakeholders interacting with EMA.

Figure 1: Regulatory lifecycle of medicines and involvement of patients/consumers (orange bubbles) and healthcare professionals (green diamonds)



Section 1 of the annual report is dedicated to areas of common interest and describes topics relevant to all stakeholder groups, whereas descriptions of the specific work of patients/consumers and healthcare professionals can be found in more detail in Sections 2. and 3. , respectively.

1.2. Framework for stakeholder relations management

In June 2016, the EMA Management Board adopted the [framework for stakeholder relations management](#), a high level document that outlines the overarching principles for managing EMA's key stakeholder interactions. The framework builds on the Agency's experience in interacting with stakeholder associations representing patients and consumers, healthcare professionals, the pharmaceutical industry and, more recently, academia. The aim of this overarching framework is to streamline interaction activities across the various stakeholder groups and align working methodologies where possible. This overarching framework will be reflected in future revisions of the individual frameworks.

1.3. Consultation methodologies

Involvement of experts, in this case patients, consumers and healthcare professionals, in EMA activities is not a 'one size fits all' methodology. The particular method used must fit the activity and the outcome that is desired. The Public Engagement department has developed various techniques for involving stakeholders (Table 1).

Each activity must be considered on a case-by-case basis and the best technique decided for that particular activity i.e. is a one-to-one discussion best or could we request our experts to reach out to their community to gain broader input on the particular issue? The objective is to have different options available to meet the requirements as needed for the issue being discussed. Having various methodologies in place also provides the option to use a combination of methodologies such as a written consultation for a larger group combined with the attendance of 1-2 experts to a committee meeting.

Table 1: Consultation methodologies for patients, consumers, healthcare professionals and academics in EMA activities related with evaluation of medicines

Consultation methodologies (with patients, consumers, healthcare professionals and academics)	Numbers consulted	Duration of activity	Confidential (Y/N)	Other information
Participation in scientific committee meetings (e.g. oral explanations)	1-2*	Duration of discussion: 1-2 hours	Y	Two-way discussion related to specific medicines evaluation
Participation in working party meetings (e.g. Scientific Advice Working Party)	1-2*	Duration of discussion: 1-2 hours	Y	Two-way discussion related to specific medicines evaluation
Participation in Scientific Advisory Group meetings (SAGs) or Ad-Hoc Expert group meetings	1-6*	Duration of meeting: 4-5 hours	Y	Full participation in meeting as other experts related to specific medicines evaluation
Participation in dedicated meeting on medicine or disease-specific issues	Up to 25 participants	1-2 hours	N	Two-way focused discussion on specific topic (proposed by EMA or requested by concerned groups)
Participation in written consultation (medicine /disease related)	5-30 (depending on topic)	2 weeks	Y	Written feedback only
Participation in online survey (non medicine-related)	5-50 (depending on topic)	2-3 weeks	N	Written feedback only

1.4. 10th anniversary of Patients' and Consumers' Working Party

The Patients' and Consumers' Working Party (PCWP) celebrated its 10th anniversary in 2016 with a series of events including an anniversary meeting on 14 June, interviews with previous co-chairs, video comments and written articles from past and current members and a panel discussion to launch the commemorative webpage.

The anniversary [meeting](#) provided an opportunity to reflect on how the involvement of patients in EMA

activities has evolved since the creation of the Agency and discussed future priorities and challenges.

A panel discussion with members of the PCWP took place during the meeting with all eligible organisations on November 30 and was the occasion to launch the [webpage](#) commemorating the 10th anniversary.

EMA created the PCWP following the success of the Working Group with Patients Organisation and the adoption of the [framework of interaction](#) in 2006. Key achievements of the working party based on recommendations made to EMA include:

- Publication of information on medicines addressed to the general public. These documents are reviewed by patients to ensure that the information is clear and relevant.
- Determination of eligibility criteria for patient and consumer groups working with the Agency;
- Contribution to the involvement of patients as experts in regulatory activities.
- Implementation of patient recommendations in the revision of the Pharmacovigilance legislation and involvement of patients as members in the PRAC.

In addition to the main highlights of the 10th anniversary meeting, the webpage has an interview with the first co-chairs of the working group (Isabelle Moulon and Frits Lekkerkerker) as well as reflections by members and highlights and challenges of the first 10 years as described by the original members.



1.5. Working parties

1.5.1. Elections

2016 also marked the end of the term of the co-chairs of both the healthcare professionals' working party (HCPWP) and the patients' and consumers' working party (PCWP).

The HCPWP elected Gonzalo Calvo as its co-chair at the September 2016 meeting for a second mandate. Professor Calvo, a physician by education and consultant in clinical pharmacology in Barcelona, Spain, has extensive experience in medicines regulation, including nearly ten years as a member of EMA's Committee for Medicinal Products for Human Use (CHMP).

"My aim is to consolidate the work initiated and open new paths to strengthen the role and impact of healthcare professionals in regulatory activities, securing fast and safe access to new medicines," said Professor Calvo. "Close collaboration with the patients and consumers' working group has been instrumental for the progress of HCPWP work. I do not see any other way forward than enhancing our interaction with patients and consumers."

- Gonzalo Calvo



The PCWP elected Kaisa Immonen as its new chair. Kaisa Immonen holds a Master of Arts (MA) in international relations and conflict analysis from the University of Kent in the United Kingdom. She has eight years' experience in European Union health policy gained firstly at the Thalassaemia International Federation, a rare-disease patient organisation in Nicosia, Cyprus and then with the European Patients' Forum (EPF), in Brussels, Belgium.



"The PCWP in its ten years of existence has been a catalyst for a profound culture change at EMA and to some extent at national agencies. Our challenge now is to take the involvement of patients and consumers in the Agency's work even further. As co-chair, I will bring a broad, cross-disease patient perspective to the role, as well as an in-depth knowledge of all the regulatory and policy frameworks relevant to the work of EMA."

- Kaisa Immonen

1.5.2. Mandates

The mandates for the PCWP and HCPWP member organisations were renewed. All organisations who wanted to re-apply for membership needed to fulfil the revised eligibility criteria adopted in 2014 (described in the Annual Reports 2014 and 2015). The EMA decides on membership of the working parties based on the organisations appropriateness to the subjects covered within the scope of the working party's mandate.

There is a maximum of 20 member organisations per working party. June 2016 marked the end of the current 3 year mandate for both PCWP and HCPWP and the new mandate spans 2016-2019. For the HCPWP it was the first renewal and the Agency will thus aim for continuity of the composition of the working party. For the PCWP it was the fourth renewal and in this case, members who wanted to renew or apply were asked to provide a motivation letter describing their involvement with the working party and EMA to date as well as their anticipated commitment to the working parties work for the next three years. The renewal procedure was organised between April - May 2016 and the Executive Director's Decision was finalised by 31 May 2016. A complete list of [PCWP](#) and [HCPWP](#) working party members has been published.

1.5.3. Work plans for the working parties

The working parties define their work plans annually (for 2017: [PCWP](#) and [HCPWP](#)) however in addition in 2016 in line with the new mandate (2016-2019), a [global view](#) encompassing the term of the mandate was developed. The work plans for the working parties align with the [EU medicines agencies network strategy to 2020](#) and the [EMA multi-annual work plan](#) and focus particularly on theme 1 'contributing to human health' and theme 3 'optimising the operation of the network'.

In addition to these longer term objectives, the core activities remain unaltered and these activities are reflected in the pages of these reports.

1.6. Implementation of eligibility criteria for organisations working with EMA

As described above, the eligibility criteria were revised and 2016 saw the implementation of these criteria. A total of 65 eligible organisations were reviewed. For a complete list of eligible organisations, please see Table 10 and Table 21.

1.7. PCWP and HCPWP joint meetings

The joint working party meetings cover subjects that are of interest and relevance to all stakeholder groups (patients, consumers, healthcare professionals). Each working party has an observer from the other working party as part of its membership; however it is also important that the majority of topics are discussed together with both working parties.

In addition to the patient, consumer and healthcare professional members of the working parties, representatives of EMA scientific committees, including the CHMP, COMP, PDCO, CAT, PRAC and HMPC, are also invited to provide brief updates on their committees' activities.

During 2016, two PCWP and HCPWP joint meetings were organised ([9 March](#), [20 September](#)) and agendas and minutes of the meetings along with the presentations are available. Summaries of a selection of topics discussed are provided below.

1.7.1. Working party topic groups

Topic groups on specific subjects with defined terms of reference and mandates were launched and extensively described in the [annual report \(2015\)](#). Some topic groups achieved their objectives and were closed, with the option to reactivate them if and when needed, others are ongoing and adapting and refining their scope.

A separate [report](#) has been published with all recommendations and conclusions from the various topic groups.

1.7.2. Member's voice

This section was introduced into the agendas of the working party meetings in 2015 as part of the initiative to encourage contributions from working party members in the context of learning from each other. Member's voice is now a standard part of the agenda of the joint working party meetings. During the course of 2016, six organisations presented their initiatives to the group; one example from a patient group was a presentation by EFA of a health literacy initiative for young patients with asthma to promote adherence to treatment and from the healthcare professional side, PGEU highlighted the launch of The European Pharmacists' Professional Forum (EPPF).

1.7.3. Update to Eudravigilance database for users

New Eudravigilance website enhancements were [presented](#) during the joint working party meeting in March. [Eudravigilance](#) is the system for managing and analysing information on suspected adverse reactions to medicines that have been authorised in the European Economic Area (EEA). The EMA operates the system on behalf of the EU medicines regulatory network. This portal allows users to view the total number of individual suspected side effect reports submitted to EudraVigilance for each centrally authorised medicine and these can be filtered by age group, sex, type of suspected side effect and outcome.

The enhancements will allow the general public to view the number of cases received over time and provides the possibility to download data using various criteria (age, sex, time, geography) in an excel file for further analysis. A reminder was made to use the existing www.adrreports.eu portal for any suspected adverse reaction data queries.

1.7.4. Personalised medicine

In a [presentation](#) during the September joint meeting, personalised medicines were described and the areas where patient and healthcare professional input would be of highest value were emphasised. With respect to patients' priorities and their role in interventional trials with personalised medicines, what has changed with the advent of -omics and how are they perceived, what are the needs and implication. Whereas for healthcare professionals, a consideration of their role in clinical research and improving the interface with research communities was described.

Patients and healthcare professionals would be invited to support the development of clinical data gathering tools as well as to contribute to the evaluation of the impact personalised medicines on public health along with other stakeholders.

A workshop on Personalised Medicine will be organised in 2017 in the context of the joint working party meeting. A report will be published following the event.

1.8. Workshops and Information Sessions

1.8.1. Information session on communication and information on medicines

This information session, organised as part of the work of the PCWP and HCPWP, took place on 8 March 2016. The objective was to promote open discussion on the needs and expectations of patients and healthcare professionals and to provide clear guidance to experts in the field of communication and information on medicines that could support future advancements.

The [day](#) began with an overview of the current situation presented by speakers representing regulators, medicines information bulletins, pharmacists, academia and the European Commission. Breakout sessions gave participants the chance to discuss opportunities and challenges in the production, dissemination and use of information about medicines in greater depth. Areas for future research were further discussed before bringing together the various strands of the day's considerations to define key messages and focus areas for future work. A [report](#) of the information session is also available.

1.8.2. Social media workshop

A joint PCWP/HCPWP workshop on social media was held on 19 September 2016. The [objectives](#) of the workshop included providing an overview of what social media are and what they are being used for in the context of medicines regulation; sharing practices on how social media is used to amplify communication on medicines information and regulatory outputs and for gathering medicine-related data; reflect on how this may influence behaviours and attitudes towards medicines information and use and to identify areas requiring action from regulators, patients and healthcare professionals.

This workshop was the first in a series of meetings that will cover linked topics related to advances in digital health including social media, 'big data' analysis and IMI (Innovative Medicines Initiatives) projects such as WEB-RADR2 , where EMA will provide a platform for discussion and an opportunity for mutual learning. A [report](#) of the workshop has been published.

1.8.3. IMI - WEBRADR workshop

The second workshop of the Innovative Medicines Initiative (IMI) WEB-RADR project was held on 19 October 2016. The project aimed to deliver a mobile app for healthcare professionals and the public to

report suspected adverse drug reactions and to develop a text mining and analysis tool of publically available data to complement existing methods of safety signal detection for medicines. Patients and healthcare professionals have been involved in the project team and others from these stakeholder groups have been consistently consulted.

This [second workshop](#) included 13 representatives from patients' organisations that covered areas such as cancer, rare diseases, AIDS, allergies, multiple sclerosis as well as consumers' interest groups and six healthcare professionals representing paediatrics, clinical pharmacology and urology as well as pharmacists and doctors. This workshop provided an opportunity to engage with consumers, patients, healthcare professionals and medicines regulatory authorities to discuss latest developments and to obtain input and feedback to maximise the utility and benefits of the project deliverables.

1.8.4. Workshop on identifying opportunities for 'big data' in medicines development and regulatory science

On 14 and 15 November 2016, EMA organised a [workshop](#) on big data. With vast volumes of data being generated, the way that benefit-risk of medicines is assessed has potential to be transformed. It is important for the European Medicines Agency and the European Union medicines regulatory network to gather information on the latest developments in big data from the perspective of all stakeholders in order to identify how and when the multitude of data sources may contribute to medicinal product development, authorisation and post-marketing surveillance.

Patients representing AIDS, cancer, and Alzheimer's and groups representing consumers and public health attended as well as healthcare professionals representing clinical pharmacology, hospital pharmacy, and primary care. One patient representative from the European Dravet's syndrome association presented their project and tool for data capture.

A recording of the workshop and a [report](#) are available.

1.8.5. EMA workshop on extrapolation of efficacy and safety in medicine development across age groups

This [workshop](#) on 17 and 18 May 2016 follows the publication of a [draft reflection paper](#) on extrapolation of efficacy and safety in paediatric medicine development.

The goal of the meeting was to agree on recommendations for clinicians, modellers and statisticians that should result in an explicit and systematic approach for decision making alongside the life cycle; hence optimising the chances for successful development and approval. An [outcome report](#) has been published. Patients representing European patients were present and one speaker provided the patient perspective on extrapolation and healthcare professionals representing paediatricians and paediatric gastroenterology, hepatology and nutrition also attended.

1.8.6. Multi-stakeholder advanced therapy medicinal products (ATMPs) expert meeting

EMA organised an [expert meeting](#) on 27 May with representatives from the European medicines regulatory network and the European Commission in addition to other stakeholders. The purpose of the meeting on ATMPs was to address the challenges identified to pharmaceutical innovation in Europe.

The aim is to understand the current European Union environment for ATMPs and to explore new or different ways for stimulating innovation, facilitating European research and development and accelerating patients' access to high quality, safe and efficacious ATMPs. The majority of the meeting comprised an open discussion on 'Exploring solutions together: discussion and brainstorming of

solutions for ATMPs on the following areas' where clinical haematologists and hospital pharmacists were present along with patients' groups for cancer and genetic diseases. An [outcome report](#) has been published.

1.8.7. Workshop on single-arm trials (SAT) in oncology

On 30 June, the EMA held a joint workshop with the European Society for Medical Oncology (ESMO) on single-arm trials in the field of oncology.

This [workshop](#) aimed to address situations where a medicine shows significant anti-cancer activity in patients where there is no treatment option or where conducting trials with a comparative arm is difficult such as in rare cancers or selected populations.

Workshop participants discussed experience gained, to date, with marketing authorisations based on single-arm trial data, the strengths and weaknesses of different approaches, and opportunities from data sharing initiatives.

The workshop was attended by 11 healthcare professional members of oncology specific societies as well as those representing specialised nurses and neurology. There were seven patients' representatives from myeloma, lymphoma, men's health, and all cancer groups and a presentation was made by a melanoma patient representative on clinical trial design. All [presentations](#) are available and a report will be published.

1.8.8. Joint EFGCP/DIA/EMA Better Medicines for Children Conference on Optimisation of Drug Development for the Benefit of Children

This [workshop](#) took place on 10 and 11 October and was organised by EFGCP, with the partnership of DIA and EMA.

The aim of the conference was to discuss how medicines development can be further optimised to benefit children's health. A discussion on lessons learnt in the 10 years of the EU Paediatric Regulation new concepts and strategies for an integrated approach at each and every step of paediatric development and sessions dedicated to specific areas of expertise such as neonatology as well as evolving concepts such as the approach to extrapolation from adult data.

In a panel discussion entitled 'How can children, carers and families contribute to drug development?' three young people from different countries in Europe, were invited to participate in the panel and present their viewpoint on this subject.

In addition, eight patients' representatives from Duchenne muscular dystrophy, Rett syndrome, rare diseases, and AIDS attended the meeting along with 21 members representing academic organisations. A [report](#) of the workshop has been published.

1.8.9. DIA Information Day on Medication Errors

An information day on medication errors was organised by the DIA and hosted at EMA premises on 20 October. The objective of the [workshop](#) was to raise awareness of EU pharmacovigilance obligations for medication errors and to discuss operational aspects and good practice recommendations with regard to medication error reporting, evaluation and prevention, with insight into the current regulatory thinking on how to tackle medication errors within health care delivery systems for the benefit of patient safety.

European pharmacists and rare disease patient representatives presented their perspectives on how better to engage their stakeholder groups in the session entitled 'Patient and healthcare professional engagement in error prevention strategies'.

Healthcare professional experts in the field of medication errors and patients representing immunodeficiencies, European patients and consumer groups were represented amongst the attendees.

1.8.1. Initiative for patient registries

A workshop was held on 28 October on the [initiative for patient registries](#): strategy and pilot phase that aims to facilitate interactions between registry co-ordinators and potential users of registry data both at an early stage of the development, during the marketing authorisation evaluation procedure and post-authorisation.

The workshop brought together multiple stakeholders including registry owners, industry, representatives of health technology assessment bodies and regulators to discuss the challenges and barriers to collaboration and identify specific solutions. Attendees also included eight patient representatives from Parkinson's disease, Duchenne muscular dystrophy, cystic fibrosis, AIDS and rare diseases as well as consumer interest groups. Healthcare professionals representing cardiology and academics from the London School of Economics also attended.

Following a pilot phase, the cross-committee task force, who will coordinate the pilot in collaboration with EMA, will compile a 'lessons learnt' from the case studies that will lead to recommendations for facilitating the conduct of registries.

1.8.2. Measuring the impact of pharmacovigilance activities workshop

In the context of the adoption of a [strategy](#), by the Pharmacovigilance Risk Assessment Committee (PRAC), for measuring the impact of pharmacovigilance activities performed at EU and Member States level, a [workshop](#) was organised on December 5 and 6 at EMA.

Pharmacovigilance activities include risk management planning and the detection, assessment, evaluation and management of drug-related adverse effects and the aim of this workshop was to facilitate the implementation of the PRAC strategy with a particular focus on the development of methodologies and the fostering of collaboration.

In a session entitled 'Way forward and next steps' the patient and healthcare professional speakers presented their respective perspectives on the how data can be generated on behavioural changes in light of pharmacovigilance activities. All [presentations](#) from the workshop have been published.

1.8.3. Adaptive pathways workshop

A [workshop](#) was organised by EMA in collaboration with the European Commission following the publication of the [final report](#) on the pilot.

During the course of the pilot, numerous concerns were raised by civil society and were distilled into 10 recurring topics. To prioritise these topics for the workshop, the PCWP/HCWP were consulted and ranked their top five concerns ([Briefing book](#)).

Participants representing patients, consumers and healthcare professionals were invited to present their perspectives in the first session of the workshop addressing patients' needs.

The attendees include 18 consumer and patients' representatives from multiple sclerosis, cancer, Duchenne, thalassaemia, Rett syndrome, rare and genetic disease groups as well as healthcare professionals representing cardiology, endocrinology, oncology, clinical pharmacology, hospital pharmacists. Academics from the United Kingdom, United States, Crete, Netherlands and Japan also demonstrated the broad interest of this topic. A summary [report](#) of the meeting is available.

1.9. Increasing understanding and awareness of EMA activities

1.9.1. Information on EMA website

The webpages of the EMA website provide useful information regarding its activities and current events. A feed of all of the Latest News is provided on the home page and access to specific landing pages for 'Patients and Carers' as well as 'Healthcare Professionals' can be accessed under the 'Find information for...' section. These are also fed with relevant articles on a permanent basis and the Featured Information is updated quarterly. A similar section for 'Academia' will be launched in 2017.

1.9.2. Partners and networks web pages

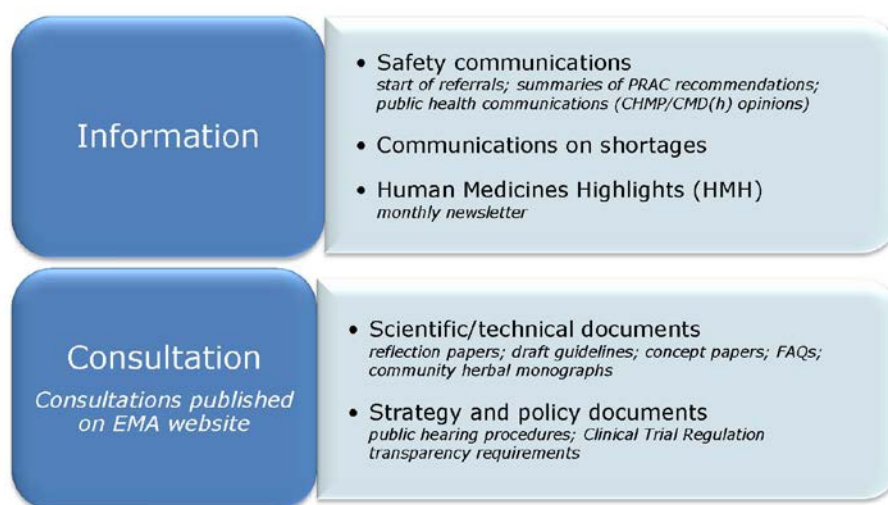
Healthcare professionals and Patients and Consumers have dedicated pages within the Partners and Networks section of the website that provides information on Agency activities where patients and consumers are involved, how they can get involved, which organisations are currently involved with the EMA as well as training and supporting key documents for these activities.

These webpages have been updated and describe the history of the interactions of EMA with the stakeholder group, the formal basis for the interactions (Frameworks of interaction) and details the different activities where patient or healthcare professionals are involved at EMA. There are links to useful documents as well as links to other relevant pages on the website. Specific pages supporting the work of Academics and Researchers will be developed in 2017.

1.9.3. Targeted dissemination of information: role of organisations

The EMA recognises patients', consumers' and healthcare professionals' organisations as key facilitators to communicating with the wider community. Information produced by the Agency is sent to stakeholders for consultation and feedback as well as to cascade to their organisations (Figure 2).

Figure 2: Targeted dissemination of information with EMA stakeholders



Through the internal stakeholders' database, comprising European and international organisations, the Agency has disseminated and encouraged further cascading of over a hundred documents in 2016, including:

Safety communications

Safety communications provide information from safety reviews by the Agency's Pharmacovigilance Risk Assessment Committee (PRAC), which is responsible for the assessment and monitoring of human medicines. Safety communications also include information on shortages.

- summaries of PRAC recommendations
 - high-level summaries of the PRAC recommendations on a specific safety/efficacy concern
- public health communications
 - documents that describe EMA recommendations following safety/efficacy concerns over medicines already on the market;
 - published at time of PRAC/CHMP opinion /CMDh position
- information on shortage of medicines
 - information on medicine shortages that affect or are likely to affect more than one EU Member State, where EMA has assessed the shortage and provided recommendations to patients and healthcare professionals (via DHPC¹);

Scientific guidelines, reflection papers, concept papers, questions and answer documents, EU herbal monographs released for public consultation

- The Agency develops **scientific guidelines** in consultation with regulatory authorities in the European Union (EU) Member States, to help applicants prepare marketing-authorisation applications for human medicines. Guidelines provide a basis for practical harmonisation of how the EU Member States and the Agency interpret and apply the detailed requirements for the demonstration of quality, safety and efficacy that are in the Community directives.
- **Concept papers** are documents prepared by a European Medicines Agency working party prior to the drafting of a guideline, setting out the problem, the scope of the work, the resources needed and the timeframe.
- **Reflection papers** are developed to communicate the current status of discussions or to invite comment on a selected area of medicine development or on a specific topic. A reflection paper does not provide scientific, technical or regulatory guidance, but may contribute to the future development of such guidelines or related documents.
- The EMA develops "**Questions and answers**" or "Frequently asked questions (FAQ)" documents to provide additional public information on topics of particular interest. They are intended to briefly communicate, in easily comprehensible language, requirements, practices or interpretations responding to the most frequent questions in a specific area.
- **Herbal monographs** comprise the scientific opinion of the Committee on Herbal Medicinal Products (HMPC) on safety and efficacy data concerning an herbal substance and its preparations intended for medicinal use.

¹ DHPC: Direct Healthcare Professional Communication

Strategy and policy documents released for public consultation

- When applicable, the Agency releases draft strategy and policy documents for public consultation and interested parties are invited to review the proposed draft rules and send their comments. Following review of all comments, the Agency will present the final rules of procedure to its Management Board for adoption. After that, they will become operational.

For all these documents, a targeted email is sent to a selection of organisations that has expressed an interest in the therapeutic area or topic related to the communication. In each email, the Agency kindly requests the original recipients to further disseminate the information to any other parties who might be interested. As such, the organisations act as a multiplier of information published by the Agency.

Human Medicines Highlights (HMH); a monthly newsletter addressed primarily to organisations representing patients, consumers and healthcare professionals. It provides a summary of key information relating to medicines for human use published during the previous month by the EMA. Information is selected based on recommendations from consulted patients, consumers and healthcare professionals. During 2016, 12 issues were published and are available in the News and Events tab on the homepage under the [Newsletter](#) heading.

1.9.4. Resources pages

The Resource pages for both patients and healthcare professionals have been updated to include useful links to relevant workshops as well as the 'videos' that provide bite-sized information on EMA activities and interactions with its stakeholders entitled 'EMABasics'. The list currently includes videos and related documents or pdf versions of the slides with text (Table 2).

Table 2: EMABasics videos and presentations

'EMA Basics' videos	Related documents
The European Medicines Agency	Presentation - The European Medicines Agency
The centralised procedure	Presentation - The centralised procedure
Involvement of patients	Presentation - Involvement of patients
The Patients' and Consumers' Working Party	Presentation - The Patients' and Consumers' Working Party
EMA video for patient representatives	Involvement of patient representatives in scientific advice procedures at EMA Involvement of patient representatives in scientific advisory groups at EMA
Pharmacovigilance	Presentation - Pharmacovigilance
How EMA works with healthcare professionals	Presentation - How EMA works with healthcare professionals
Scientific advice: what to expect and how to prepare	Presentation - Scientific advice: what to expect and how to prepare
Declarations of interests: a practical guide	Presentation - Declarations of interests: a practical guide
How patients are involved in the review of documents	Presentation - How patients are involved in the review of documents
What is a European safety referral	Presentation - What is a European safety referral (New)

1.9.5. External queries

Every year the Agency receives external queries from individuals through the online information request form on the EMA website. In 2016, the Agency responded to 513 queries from patients/consumers, 207 from healthcare professionals and 226 from academia/research institutes.

Approximately 21% of the queries were received from non-EU countries. Most of the questions were related to the availability of medicines and adverse effects.

1.10. Contribution to EMA transparency initiatives

1.10.1. Clinical data publication

The EMA began to publish clinical data submitted by pharmaceutical companies to support their regulatory applications for human medicines under the centralised procedure in October 2016. This is based on EMA's policy on the publication of clinical data (also known as Policy 070).

The purpose of publishing clinical data is to avoid duplication of clinical trials, foster innovation and encourage development of new medicines; build public trust and confidence in EMA's scientific and decision-making processes; and to help academics and researchers to re-assess clinical data.

User testing

A user test of the prototype of the [EMA's clinical data website](#) took place in February 2016. Feedback was implemented and a second user test was conducted in July over a two-day period. The feedback was largely positive from both the patients and healthcare professionals who were involved. Healthcare professionals represented a clinical pharmacology and therapeutics organisation and hospital pharmacists and patients were from rare diseases, primary immunodeficiencies, Alzheimers and consumer groups.

1.11. Input on EMA pharmacovigilance-related initiatives

1.11.1. Interactions with PRAC civil society representatives in preparation for future Public Hearings

In 2016, several meetings were held with the Public Engagement department and the PRAC topic group on patient engagement on the subject of preparing for public hearings.

The new pharmacovigilance legislation lays down provisions for the PRAC to hold public hearings on safety reviews of medicines. In anticipation of a future public hearing, the EMA conducted a 'dry run' to prepare the PRAC, test the logistics (e.g. invitations, movement of the public in the building, broadcast etc.) using a fictitious medicine and Agency staff to play the role of the public. The PRAC topic group together with EMA prepared a specific guidance document for the committee. The decision to hold a public hearing will be decided by the PRAC on a case by case basis.

1.11.2. Pharmacovigilance legislation: tenth stakeholders forum

On 21 September 2016, the [tenth stakeholder forum](#) on the Pharmacovigilance legislation took place.

Representatives of patients and consumer groups as well as healthcare professional organisations were invited to participate in an open panel discussion. A representative of the Pharmaceutical Group of the European Union – an EMA eligible organisation presented on the actions arising from the 2015 [workshop](#) on risk minimisation measures.

In addition, ten patients representing older people, cancer, neurological associations, multiple sclerosis, rare diseases, myeloma, European and international patients as well as consumers' interests were also present. Healthcare professionals also attended and the eight members represented organisations such as standing committee of European doctors, clinical pharmacologists, hospital pharmacists, specialist nurses, gastroenterologists, rheumatologists and primary care.

The [agenda](#) and all [presentations](#) as well as a recording of the meeting have all been published.

1.11.3. Measuring the impact of pharmacovigilance activities workshop

While the topic of this workshop also belongs in this section it is described in more detail in the workshop section 1.8.2.

1.12. *Involvement in Networks and research projects*

The EMA is involved in several research projects in varying capacities. Where possible and increasingly so, patients and healthcare professionals are invited to participate as partners, in steering groups etc.

1.12.1. European Paediatric Research Network (Enpr-EMA)

The eighth annual [workshop](#) of Enpr-EMA was held in June and the first day of the workshop open meeting to all stakeholders, including patient and parent organisations, network representatives, pharmaceutical industry staff responsible for paediatric studies and regulators. The main topics of the workshop were the deliverables of the working groups and the implementation of the new Clinical Trial Regulation with a focus on ethics related issues. A [report](#) is available. A member of the PCWP is a member of the coordinating group of Enpr-EMA.

1.12.1. European Network of Centres for Pharmacoepidemiology and Pharmacovigilance (ENCePP)

The European Network of Centres for Pharmacoepidemiology and Pharmacovigilance ([ENCePP](#)) is a network of over 170 research centres, existing networks and providers of healthcare data, which is coordinated by the EMA. Patients' representatives form part of the Steering Group and the Interested Parties and Stakeholder group.

2. Interactions with patients and consumers

2.1. Introduction

Key actions of the current EMA framework of interaction of EMA with patients and consumers are described below along with the activities that have been achieved or that are ongoing.

- Maintenance of the network of EU patients' and consumers' organisations

A revision of eligibility criteria was established along with the organisations and implemented. Meetings of the working party and an annual meeting with all eligible organisations were maintained. Members of organisations were invited to workshops and included in the topic groups of the working parties. They were also kept informed of new authorised medicines via the monthly update of (HMH).

- Establishment of a pool of individual experts

In 2016, EMA expanded its internal database of stakeholders, which currently contains information on organisations, to also include individuals and their areas of interest across Agency activities. The database's main purpose is to identify patients and consumers to participate in activities of the EMA. In addition, those registering can opt to receive information in their area of interest. The Agency's first port of call when looking for patients is via their registered eligible patients' and consumers' organisations. However the increasing demand and diversity of activities requires a broader approach.

Patients can [register](#) to join the database and a question and answer document ([Q and A](#)) has been prepared.

- Participation at key milestones during the lifecycle of medicines

In addition to all the stages of medicines development where patients are already involved (Figure 1); a pilot project to involve patients directly in the benefit-risk evaluation of medicines within the oral explanations at CHMP meetings concluded in 2016 with a report to be published early 2017. Patients have also been included in key scientific advice meetings with HTA bodies and regulators.

- Building of capacities of patients and consumers invited to participate in EMA activities

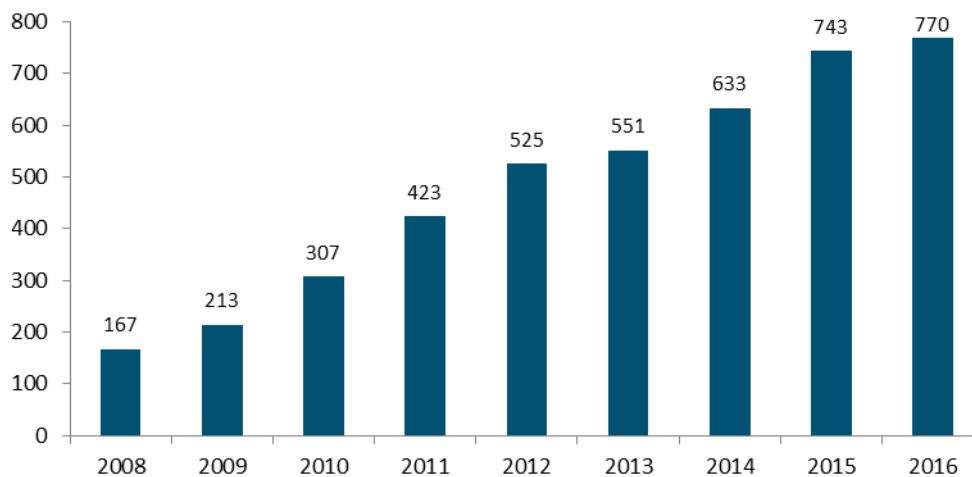
In order to ensure that patients and consumers involved in EMA activities are prepared and supported for their involvement, training is offered by the Agency (Section 2.3.). In addition to a one-day training held annually, a series of online videos and explanatory summary sheets have been progressively developed (see section 1.9.4.) and are available on the patients and consumers webpages to support the continual learning, not only of those invited to be involved with EMA but also for the public who may want to learn more about how the Agency works.

- Increasing transparency on the involvement of patients and consumers and their organisations in Agency activities.

One of the major priorities of both EMA and patient/consumers involved with the Agency has been to ensure transparency of decisions and activities. Transparency will always be extremely important because it increases trust in and understanding of regulatory processes. This is a mutual agreement and changes to the eligibility criteria, described in the 2015 annual report, resulted in a requirement for EMA eligible organisations to publically declare all sources of funding on their website.

The overall number of cases of patient/consumer involvement during 2016 is shown below in Figure 3. Despite an expectation of a plateau in the volume, they have continued to rise, reflecting not only a sustained involvement of patients in EMA activities but also an increased demand linked to demonstration of the added-value of patients in the activities where they are involved.

Figure 3: Overall number of patient and consumer involvement in EMA activities (2007-2016)



2.1.1. Framework for interaction with patients and consumers

Specific actions that are ongoing or completed, relating to the action plan of the current framework for interaction with patients, consumers and their organisations are described in Section 2.1. This will be reviewed and updated during 2017.

The overarching framework (section 1.2.) will be reflected in future revisions of the framework of interactions with patients and consumers and their organisations.

2.2. Patients/consumers in EMA activities and scope of representation

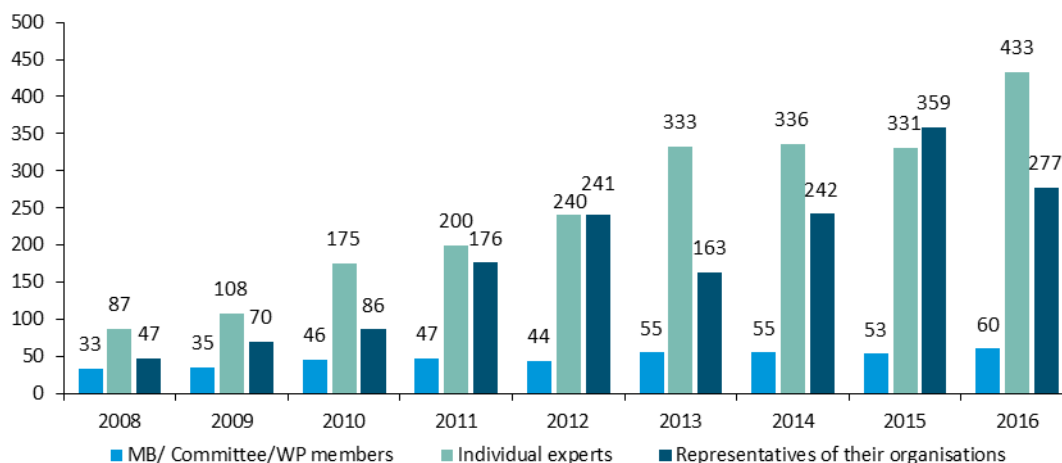
Patients and consumers are involved in a diverse array of Agency activities either as representatives of all patients’ organisations, representatives of their own organisations or as individual patient experts. Figure 4 shows the different activities associated and the scope of their representation.

Figure 4: Patients/consumers in EMA activities and scope of representation



Figure 5 shows the distribution of the numbers of patients involved in the categories as mentioned above. More detail about each of these activities is provided in the corresponding sections below.

Figure 5: Overview of individuals involved in EMA activities (2007–2015)



2.2.1. Patients representing patients' organisations

2.2.1.1. Membership in EMA management board and scientific committees

As described in Figure 4, patients involved in the EMA Management Board and the Scientific Committees serve to represent patients' organisations. These members are appointed by the European Commission in consultation with the European Parliament on the basis of their expertise. All members are required to have signed a Declaration of Interest and Confidentiality form as do all experts involved in activities in the Agency.

Management Board: The Management Board is the Agency's integral governance body and includes two members representing patients' organisations. This group has a general responsibility for budgetary and planning matters, the appointment of the Executive Director and the monitoring of the Agency's performance.

At its June meeting, the European Medicine Agency's (EMA) Management Board welcomed new representatives of civil society who joined the Board as members. Ilaria Passarani, Head of the Food and Health Department at the European Consumer Organisation (BEUC), and Yann Le Cam, Chief Executive Officer and co-founder of the European Organisation for Rare Diseases (EURORDIS), representing patients' organisations.

Scientific Committees: There are six scientific committees for human medicines at the EMA and patients are full voting members of four of these. In this context they represent patients or patients' organisations. Activities performed by patients' representatives in these committees include orphan designation of medicinal products, assessment of paediatric investigation plans, classification of advanced therapies and assessment and monitoring of safety issues of medicines.

Table 3: Membership of patients and consumers in EMA Management Board and Scientific Committees

EMA Management Board and Scientific Committees	Members /alternates
Governance:	
Management Board (MB)	2
Scientific Committees:	
Committee for Orphan Medicinal Products (COMP)	3
Paediatric Committee (PDCO)	3 / 3
Committee for Advanced Therapies (CAT)	2 / 2
Pharmacovigilance and Risk Assessment Committee (PRAC)	1 / 1
TOTAL	17

2.2.2. Patients/consumers representing *their* organisations

2.2.2.1. Membership of Patients' and Consumers' Working Party (PCWP)

In addition to these activities, patients are also members of two working parties of the EMA, in particular the Patients and Consumers Working Party (PCWP) where there are currently 19 members and 21 alternates or observers (Table 4). The working party is co-chaired by one member of the working party, currently Kaisa Immonen (BEUC), a representative from a consumer's organisation and one representative from EMA, currently Isabelle Moulon.

Four patient representatives are also members (on a rotational basis) on the Healthcare Professionals Working Party to observe and introduce the patient perspective where necessary.

Table 4: Membership of patients and consumers in EMA working parties

Membership of working parties (WP)	Members /alternates (observers)
Patients and Consumers Working Party (PCWP) + co-chair	19+1 / 21
HealthCare Professionals Working Party (HCPWP)	2
TOTAL	43

The PCWP is an important platform for exchange between the Agency and patients' and consumers' organisations. Discussions occur on a wide-range of topics that are of direct or indirect interest to patients in relation to medicinal products. This working party collaborates and holds common meetings with the Healthcare Professionals Working Party (HCPWP) (see Section 1.7.).

In addition, the PCWP also met on:

- 14 June was the first meeting within the new 2016-2019 mandate. The morning session consisted of an overview of the composition of the working party, a summary of the type of activities members will be involved in, as well as showing relevant information located on the EMA website. Updates on the PRIME and adaptive pathways initiatives were provided as well as further information on the proactive publication of clinical study reports. The second half of the day was dedicated to the 10th anniversary of the PCWP (see 2.2.2.2.)
- [29 November](#) - Training session – described further in Section 2.3.
- [30 November](#) – Annual meeting with all eligible organisations that ensures that all organisations are up to date with information and can also feedback to the Agency during this face to face meeting. Topics for discussion included an overview of the next steps for public engagement

including EMA readiness for public hearings, updates on HTA cooperation at the EU level, enhancements of the adverse drug reaction reporting websites and the use of genomics in clinical studies and pharmacovigilance.

In addition to these annual meetings, the EMA maintains communication with its stakeholders via email, dedicated pages on the website, newsletters, tweets and targeted communication.

2.2.2.2. 10th anniversary of the PCWP

The PCWP was established in 2006 following endorsement by the EMA Management Board of the framework of interactions between EMA and patients and consumers and their organisations. This formalised the interactions of the Working Group with Patients Organisations as well as dialogue that had been ongoing with patients and consumers since the creation of the Agency in 1995.

The 10th anniversary marked an important occasion for this unique group that 'helped the Agency to mature its reflection on how to involve patients and further reach civil society and to monitor the implementation and make sure we are on the right track in the words of co-chair Isabelle Moulon.

Figure 6: The original members of the PCWP



The Public Engagement team would like to pay tribute to one of the original members of the PCWP, Dr Wim Wientjens (1937-2016) who also served as a patients' representative on the EMA Management Board (2013-2016). Wim was a dynamic and unforgettable patient advocate whose humour and energy will be missed.

The celebratory meeting was opened with comments from EMA Executive Director Guido Rasi who said that 10 years is a good amount of time to gather experience but that we need to keep going and to further enhance and explore new ways to steer not only the development of medicines but also their use, which is even more important than just having them. He emphasised again that the role the patients play is unique because no one else can comment on the quality of life and the trade-off between benefits and risks.

This was followed by a panel discussion including representatives from a patient organisation, consumer group and EMA, who detailed how their different starting points and perspectives led them to converge on the need to work together. The three former co-chairs each provided a retrospective of what they felt were the objectives for their mandate, the challenges they faced and their most memorable moments.

The next session began with an overview of the types of engagement and the various tools available for involving patients and consumers in Agency activities as well as plans for expanding these activities to include the involvement of young people in Agency consultations. Finally, the Deputy Executive Director began his address to the audience by stating that the PCWP and the broader interactions between EMA and patients had been a success story. He mentioned three important paradigm shifts that have influenced the regulatory journey since 1996:

1. Authorisation paradigm – originally the emphasis was on granting marketing authorisations. This is no longer sufficient, as other issues are critical for patients such as timely access to medicines for patients, which goes beyond marketing authorisation and includes HTA, pricing and reimbursement etc.
2. Civil society involvement paradigm – moving from informing to involving and engaging with civil society has been a natural process
3. Transparency paradigm – from transparency on the outcome of the scientific review to access to the data on which the decisions have been taken.

Perhaps we should have a more in-depth debate for the next years to come about what we think is the best role of patients and should we rethink the role of patients within the context of regulatory activities (in the overall process).

An [anniversary page](#) has been created on the EMA website that houses a collection of video links including the recording of the anniversary meeting; reminiscence between two original co-chairs Frits Lekkerkerker and Isabelle Moulon and reflections by the current working party members. A series of short contributions can also be found where the original members (Figure 6) describe their highlights and challenges of the last 10 years. Finally the coming together of patients and consumers, a clear success story was not an obvious fit in the beginning and an article has been written about the different perspectives and objectives as well as the successful subsequent collaboration with healthcare professionals upon the creation of the HCPWP.

“10 years is a good amount of experience, but we need to keep going and.... Explore new ways to address and drive the development of medicines and how to use them”
Guido Rasi, Executive Director

This special anniversary also served as an opportunity for the FDA and Clinical Trials Transformation Initiative (CTTI) to launch their new working group with patient advocacy organisations to talk about patient engagement at the [FDA CTTI](#).

2.2.2.3. Topic groups of the PCWP

The topic groups were described in detail in the [annual report \(2015\)](#) and a report will be published with all recommendations and conclusions from the various topic groups. The aim of the topic groups is to enable brainstorming in smaller groups between plenary working parties' meetings, promote further discussion on specific topics and allow better utilisation of time during the face-to-face working parties' meetings and also provides opportunities for members of other eligible organisations to join. In Table 5, an update of the status of the existing topic groups is provided and in 2017 the working parties will discuss the potential launch of new topic groups.

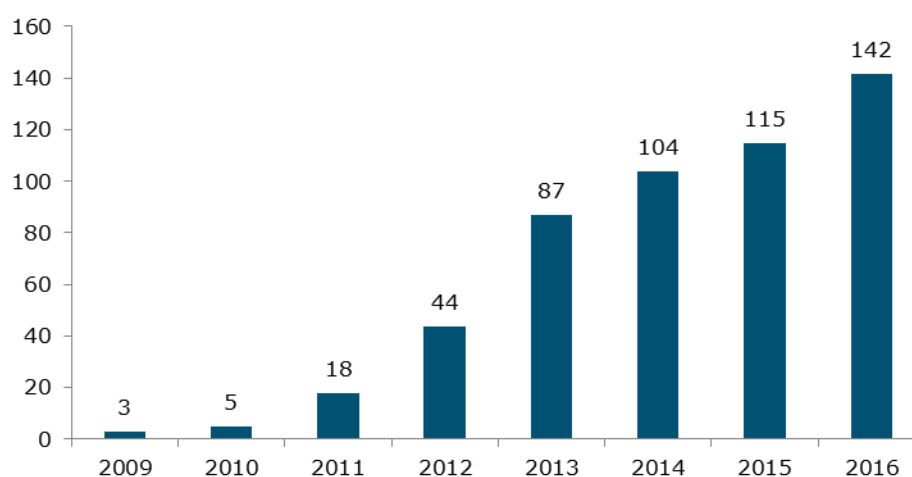
Table 5: PCWP topic groups

PCWP topic groups	Status
Measure the impact of patient involvement in EMA activities	Finalised
Acknowledge and promote visibility of patient input into the Agency's activities	Finalised
Training and support for patients involved in EMA activities	Finalised
Involvement of young people in EMA activities	Ongoing
Social media	Ongoing

2.2.2.4. Workshops, meetings and consultations

Involvement of patients and consumers in EMA organised conferences and workshops has continued to increase as the Agency endeavours to ensure that patient representatives are given opportunities to participate as often as possible; these have been described in Section 1.7.

Figure 7: Number of patients and consumers included in workshops at EMA (2009-2016)



2.2.2.5. Overview of activities involving patients' and consumers' organisations in 2016 as representatives of their organisations

Table 6 provides an overview of the different occasions and activities concerned where patients and/or consumers were involved representing their own organisation. Some of the activities described in Table 6 have been described in more detail above. For more information on other activities, please consult the [EMA website](#).

Table 6: EMA Activities involving patient and consumer organisations

Activities involving patients' and consumers' organisations	Number of representatives
Patient participation in workshops at EMA	142
Preparation by EMA eligible organisations for ongoing evaluation of criteria	36
Ad-hoc observers/experts attending PCWP meetings	32
EMA missions	16
Patients participating in EMA measuring impact of pharmacovigilance activities	10
Meetings with patients representatives of scientific committees	9
EMA consultation to PCWP (and HCPWP) on adverse drug reaction (ADR) website	3
Getting the message across — how health literacy impacts risk communication - Lunchtime talk and debate	1

EMA awareness-raising lunchtime talk	1
Observer at HCP meetings	1
TOTAL	251

2.2.2.5.1. Spinal Muscular Atrophy (SMA) workshop

A one-day [workshop](#) on spinal muscular atrophy (SMA) took place on 11 November and was co-organised by EMA, SMA Europe and TREAT NMD. The workshop brought together key stakeholders to discuss, help and advance the development of therapies for the treatment of SMA. Topics for discussion included an overview of the disease, the pharmacology of the molecules under investigation, natural history data, clinical outcome measures and biomarkers.

2.2.3. Patients/consumers as individual experts

When patients and consumers are involved in EMA activities on medicine-specific issues, they do so as individual experts. Table 7 provides an overview of the activities and number of patients and consumers as individual experts involved in the respective activities. These are further described in the text following.

Table 7: EMA activities involving patients and consumers as individual experts

Activities involving individual experts	Number of Experts
Review of documents	
Herbal summaries	29
EPAR summaries	36
Package Leaflets	60
Safety communications	26
Involvement in medicines evaluation / committee consultations	
PRAC product-related consultation	5
CHMP product-related consultation	13
CHMP consultation on labelling and package leaflet of emergency contraceptives	13
EMA/PRAC consultation on product (brochure for individuals/reminder card)	2
PRAC consultation on product related educational material	5
QRD/PRAC written consultation on risk minimisation of medication errors	3
Scientific advice/protocol assistance/scientific advice with HTA:	82
Patients attending committees' meetings as experts	8
Participation in SAGs/Ad hoc expert group meetings	28
Review of package leaflet wording (class labelling revision HIV medicines)	3
Review of urea cycle disorders medicine supply issues (Art. 31 referral)	1
Training	
Patient training day	43
Webinar on review of herbal summaries	5
Training modules from the EudraVigilance training curriculum (ADR e-learning)	6
Benefit-risk methodology project	
Conference call with Myeloma UK	1

Activities involving individual experts	Number of Experts
Focus group with Myeloma patients	10
Myeloma UK focus group	11
First conference call with Myeloma UK	1
Second conference call with Myeloma UK	1
Clinical data publication	
User testing of clinical data publication website prototype - 7 July	4
User testing of clinical data publication website prototype	4
Meetings with patients	
Meeting with patients on rheumatoid arthritis	3
EMA meeting with erythropoietic protoporphyria	18
Meeting on shortages – myeloma products	2
CHMP oral explanation (pilot project)	
CHMP Oral Explanation on Kyndrisa (drisapersen)	3
CHMP Oral Explanation on Ataluren	3
CHMP Oral Explanation on Ataluren	4
TOTAL	433

2.2.3.1. Meetings with patients

Patients' organisations can request meetings with EMA to discuss different issues concerning their involvement or with respect to medicines. In April, a group of erythropoietic protoporphyria (EPP) patients, carers and researchers in the field met with members of EMA for an in-depth exchange on the nature of the condition, the requirements, delays in the availability of treatment and an explanation of regulatory processes.

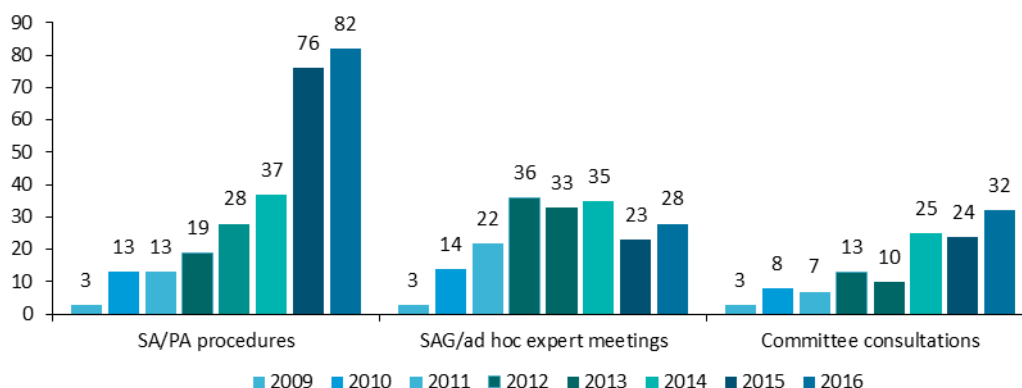
A meeting was held in June with the Hellenic society against rheumatism (ELEANA), a member organisation of the EMA eligible organisation EULAR, to discuss how the organisation and the individual patients could collaborate with EMA. Two patients and two carers met with staff of the Public Engagement Department in a brief meeting to describe processes where they could be involved.

EMA was contacted by Myeloma Patients Europe to discuss the issue of shortages of key medicines for their community. In December, three members of EMA met with two patients' representatives to discuss their concerns and activities in this area. EMA staff clarified the role and actions being taken with respect to this medicine and the patient group provided further information based on surveys being conducted in their membership. Communication regarding this issue is ongoing.

2.2.3.2. Patient and consumer involvement in scientific meetings

Figure 8 provides an overview of individual expert patient involvement in scientific procedures such as scientific advice (SA) and protocol assistance (PA), scientific advisory groups (SAG) and ad hoc expert group meetings as well as consultations by scientific committees (CHMP/PRAC). More details on each of these activities are provided.

Figure 8: Patient and Consumer involvement in EMA activities (2009-2016)



2.2.3.2.1. Input into scientific advice (SA) / protocol assistance (PA) procedures

Scientific advice provides a very good example of patient participation as well as early dialogue. The questions that can be asked by the sponsor range from non-clinical, statistics, regulatory, clinical and significant benefit, in the case of orphan designated medicines. Patients and patient representatives provide a unique perspective and their contributions can vary from providing information that results in an alteration of the advice provided to confirmation and agreement with the Scientific Advice Working Party (SAWP).

In 2016, 82 patients were involved in SA/PA procedures, either in writing and/or in a discussion meeting with the company.

In one example with a haematological condition, the patient provided both written feedback in parallel with the reports from the coordinators. This feedback was taken into account during the working party discussion and subsequently the patient was also included in the discussion meeting with the company. The patient actively participated in the discussion at the meeting and the patient's inputs were reflected in the final advice letter provided by the working party to the company. In this discussion the patient's views were very close to the coordinators which then emphasized the conclusion as this was also the patient's views in an area where no real therapy is available.

"This patient provided exceptionally valuable and knowledgeable input and participated in discussions at a very differentiated level"
Coordinator involved in Scientific Advice

In a procedure for an ophthalmological condition, the company agreed to use the endpoint proposed by the working party, which was also supported by the patient representative who attended the meeting. A discussion arose regarding exploring the further assessment of symptoms between visits using patient diaries and the patient representative indicated that it would be acceptable to fill in a questionnaire on a more frequent basis outside a visit, which could also be considered as an option.

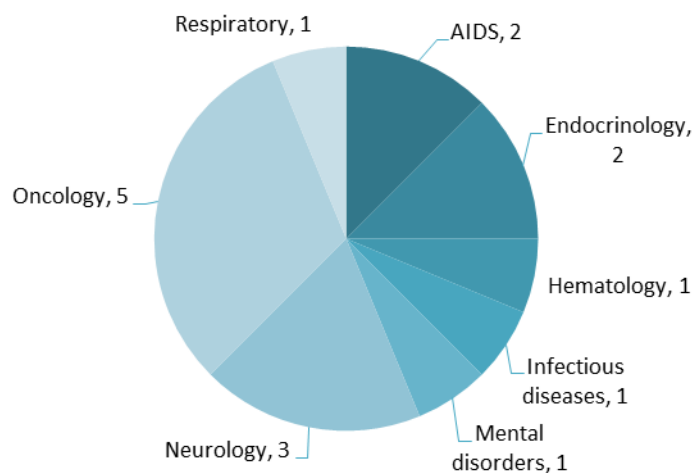
These are two examples of patient contribution to scientific advice procedures where the type of input provided varied and in one case altered the advice provided but generated further discussion in both cases.

2.2.3.2.2. Input into SAG/ad hoc expert meetings

The Agency's Committee for Human Medicinal Products (CHMP) and the Pharmacovigilance and Risk Assessment Committee (PRAC) can convene Scientific Advisory Group (SAG) or ad hoc expert group

meetings to provide advice in connection with the evaluation of a particular medicine or treatment. They consist of European experts, including patients, and they are convened on an 'as-needed' basis. Two examples below highlight the important contributions that patients make within these meetings where the patients were able to provide valuable and relevant input based on their first-hand experience and knowledge of the disorder. During 2016, 17 SAG/ad-hoc meetings (14 SAG, 3 ad hoc) covering a range of therapeutic areas (were held and patients were present in all but one).

Figure 9: Therapeutic areas covered by SAG/Ad-hoc meetings



In a SAG for multiple myeloma, the discussion focused on whether the observed benefit of the medicine, in the absence of a significant effect on overall survival, was sufficient to balance the adverse event profile. The patient representatives highlighted that the availability of a new treatment, even if associated with modest benefits and significant toxicity, is of value for patients. However they emphasised that the likelihood of experiencing unfavourable effects and the likelihood of benefit should be clearly described to allow informed treatment choice by physicians and patients, considering the available therapeutic options.

In a discussion on a treatment for rare urea cycle disorders, the focus was on what was considered the most relevant endpoint and most important evidence of efficacy. The importance of clinically relevant data on cognitive development was highlighted from the perspective of the patient representative.

When evaluating a medicine for the treatment of Duchenne muscular dystrophy, the discussion related to the differences in primary and secondary outcome effects in general and across the different subgroups. The patient representatives emphasised that differences on endpoints such as in timed function tests (TFT) that appear small and not statistically significant can be life-changing for patients. They also highlighted that future clinical trial designs should include the possibility of extrapolation of the results to a wider Duchenne population.

2.2.3.2.3. Scientific committee consultations

The Agency engages in various methods to consult with patients; scientific committees consult with patients either by inviting them to the plenary sessions as well as by written consultations. Some of the consultations are described in Table 8.

Table 8: Committee/Working party consultations with patient organisations

Committee	Subject	Contribution of patients/consumers
PRAC	Consultation on educational material	Patients' input was requested on whether the Patient Information Brochure is useful risk minimisation measure. The brochure focused on serious side effects, necessity to address them immediately, and any immune-related adverse events that may occur, persist or worsen during the treatment.
	EMA/PRAC consultation on brochure for individuals/reminder card	The patient view on the adequacy of specific educational material for risk minimisation measures was requested.
PRAC/QRD	Written consultation on risk minimisation of medication errors	Patients were consulted on further improvements for the minimisation of the risks of medication errors to ensure the safe use of diabetes medicines. There was consideration of the potential risks due to the volume of current and future medicines with the same active substances and/or fixed-dose combinations for different indications.
CHMP/QRD	Consultation with patients concerning the wording of package leaflets for HIV class medicines specifically addressing the potential risk of lipodystrophy.	Patients were requested to comment on the wording of a proposed text.
CHMP	Consultation on labelling and package leaflet of emergency contraceptives	Patient and consumer input was collected on the clarity of the proposed wording of the packaging and package leaflet.
	CHMP Oral Explanation on Kyndrisa (drisapersen) - readers guidance	Patients involved in the CHMP pilot project.
	CHMP Oral Explanation on Ataluren	Patients involved in the CHMP pilot project.

Patient involvement at the CHMP

The pilot project for involvement of patients in CHMP plenaries began in September 2014 and concluded in November 2016. During this time, patients were involved in six oral explanations at the CHMP.

The added value of including patients' perspectives within EMA benefit/risk considerations has been demonstrated many times. Patients are systematically involved in benefit-risk evaluations within SAG/ad-hoc expert group meetings, other committee consultations and scientific advice procedures. It was felt this could be expanded further directly within the CHMP and would 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 was initiated whereby patients were invited to participate in benefit-risk discussions at CHMP during specific oral explanations.

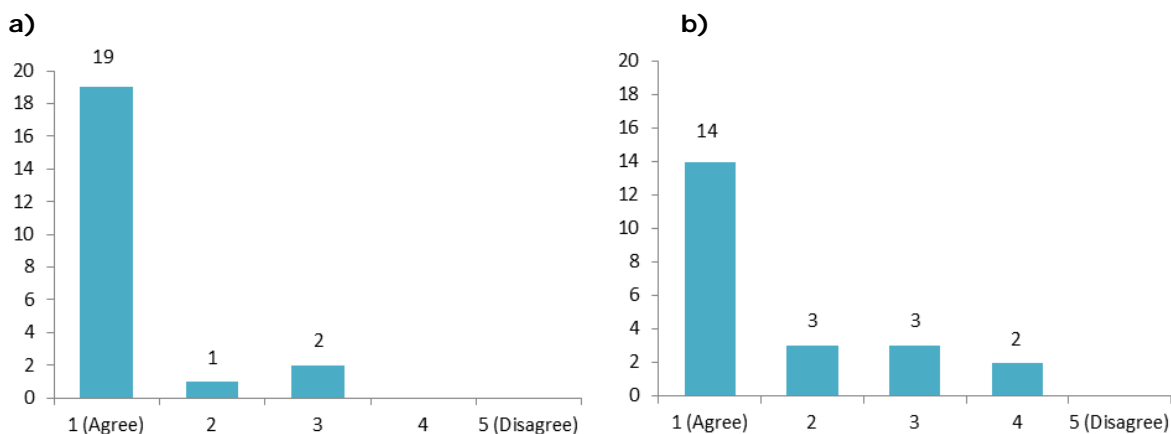
The decision to invite patients to participate was made on a case-by case basis where it was anticipated that their involvement would bring added-value to the discussion. Generally two patients (or carers) were invited, selected depending on relevance of their experience/knowledge of the particular disease/condition under evaluation; and after assessment of any competing interests. They were accompanied by a 'mentor' (PCWP member) and in addition they received personal support

(guidance on the work of the EMA/CHMP, the issues for discussion & clear definition of their role). Patients gave their views and participated in the discussions; including asking questions to the company but they do not take part in any voting process.

After each case, questionnaires were sent to the 22 regulators (rapporteurs for the products being discussed and EMA staff involved in the evaluation) and to the 14 patients/carers who participated.

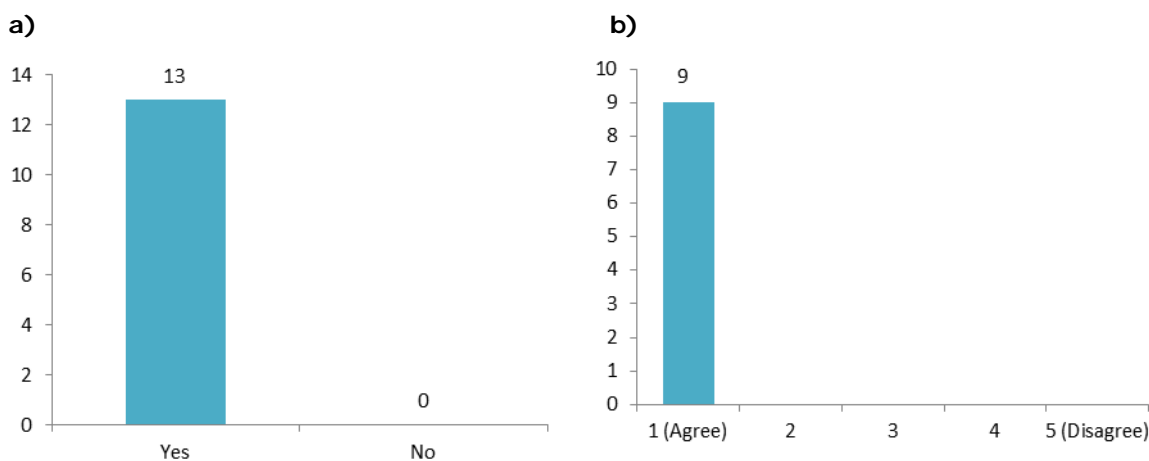
Results of the questionnaires (Figure 10) show that the majority of regulators felt that the patients were a) knowledgeable in the condition being discussed and b) that their presence in the meeting was beneficial.

Figure 10: Feedback from regulators on patient participation at CHMP plenary meetings



The patients/carers all felt that they had been appropriately prepared for the meeting and as seen in Figure 11 a) felt that they were given adequate opportunities to ask questions and provide input. Of those who answered, (Figure 11 b)) they all felt that their comments were taken into account during the discussion. One potential explanation for the lack of response on this second question by all participants is that they may not have yet received feedback on the procedure at the time of responding to the questionnaire.

Figure 11: Feedback from patients/carers on their experience at CHMP plenary meetings



These results were presented to the CHMP and 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 to 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 outside of plenary meetings and not limited to oral explanations and feedback can be obtained from a larger number of patients. Elicitation of patient preferences is also another patient engagement methodology which the committee and the EMA are 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 discussion on and evaluation of the benefit and risk of the medicine.

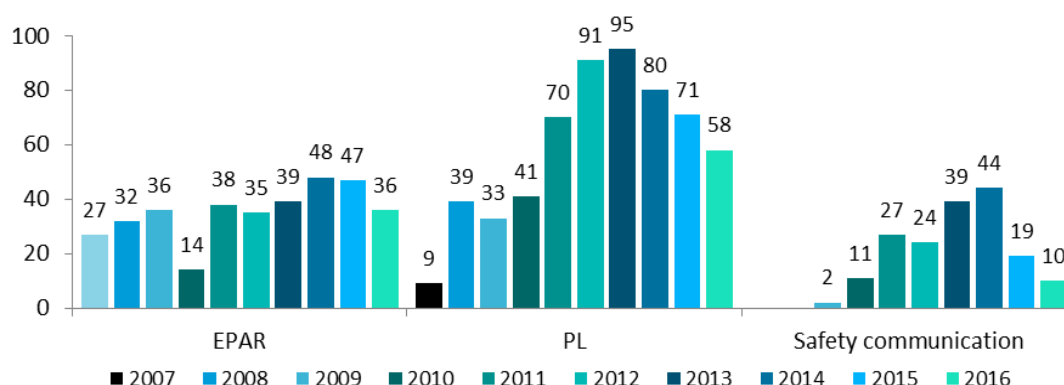
2.2.3.3. Review of EMA information

The evaluation of a medicine understandably generates many documents regarding the various aspects of its review. In the context of transparency, the EMA makes this information public via its website and also creates documents that are tailored to patients that are reviewed by patients and consumers to ensure the readability of the document. These documents include:

- The **Package leaflet (PL)** is supplied to the patient in the package in which the medicine is contained, and provides information related to the use of the medicine.
- The **European Public Assessment Report (EPAR) summary** is a lay-language document, which provides a summary of the grounds on which the EMA/CHMP based its recommendation for the medicine to receive a marketing authorisation.
- **Safety communications** refer to documents that are specifically addressed to the public on authorised medicinal products and that convey an important (emerging) message relating to the product (e.g. a product is withdrawn or suspended for safety reasons, has a new contraindication or warning, or there is a product defect).
- **Herbal summaries** are summaries of the scientific conclusions reached by the Committee on Herbal Medicinal Products (HMPC) on the medicinal uses of herbal medicines. The HMPC conclusions are taken into account by EU Member States when evaluating applications for the licensing of herbal medicines.

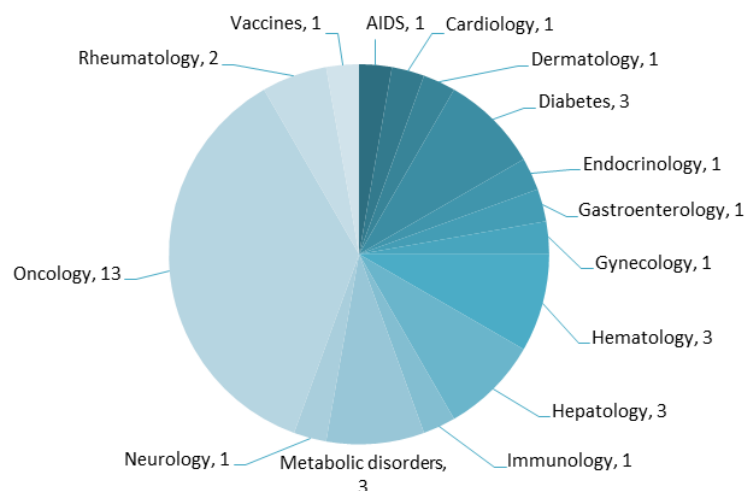
In Figure 12, the number of documents reviewed by patients and consumers is shown.

Figure 12: Documents reviewed (2007-2015): Package leaflets (PL), EPAR summaries and safety communications



In Figure 13, we see the different therapeutic areas of the EPAR summaries reviewed by patients.

Figure 13: Therapeutic areas covered by EPAR summaries in 2016



In March 2016, patients also began reviewing herbal summaries and a total of 25 summaries were reviewed by 29 patients.

2.3. Capacity-building and awareness-raising activities

Participation of patients, carers and consumers in EMA activities is supported in various ways, including training via the provision of information on the website, personalised communication and the annual training day.

One to one support: For individual patient experts invited to participate in EMA activities, one-to-one individual support and training is provided. The patients are guided through the role of the Agency and the particular procedure that they may be involved in; from scientific consultations to document review. They are directed to helpful documents and videos and supported throughout their participation from travel booking to acknowledgement of their contribution. Work is ongoing to harmonise and improve these processes between the departments in the EMA.

Annual (face to face) training day:

A second edition of the revised format of the training day was held in 2016. The feedback received from 2015 was very positive as patients felt that they gained a better understanding of what was required from them in the activities where they are invited to EMA.

Preparation prior to the annual training day includes the use of tools such as the EMABasics (see section 1.9.4.) enabling more time for exchange and discussion. Once again, a format of short presentations and breakout sessions were used to illustrate the role of patients in various activities from involvement in pre-submission and evaluation phases to post-authorisation. The breakout sessions included i) a Scientific Advice procedure, ii) Scientific Advisory Group, iii) PRAC written consultation and iv) patient review of either a safety communication or an EPAR summary. The interactions were appreciated by the attendees and the EMA staff involved in the training.

2.4. EMA awareness-raising activities

A key objective of the EMA is to raise awareness about the work of the Agency, the inclusion of patients and consumers in its activities as well as increasing general understanding of the European regulatory network activities and processes. The EMA is involved in training workshops for patients as well as in activities that raise awareness regarding patient engagement.

This involves many aspects, one of which is the participation in meetings organised by external stakeholders and these are listed in Table 9.

Table 9: EMA participation in external patients' and consumers' meetings

Promoting patient engagement	
1	EFA AGM event at UK House of Commons, London
2	Eighth European Conference on Rare Diseases & Orphan Products (ECRD 2016), Edinburgh
3	EUPATI final conference
4	EUPATI workshop on patient involvement in industry R&D, Berlin
5	EuropaBio patients bioforum, Brussels
6	FDA/Patients advocate collaborative planning conference call
7	Seventeenth world conference on lung cancer
8	Action Duchenne international conference
9	DIA Annual meeting combined with meeting with FDA, Philadelphia
10	DIA Eurometing, Hamburg, Germany
11	ECTRIMS-EMA-EMSP meeting
12	IAPO seventh patients congress, London
13	ICAN summit, Barcelona
14	IMI workshop on patient engagement strategy for innovative medicines, Brussels
Training	
15	EUPATI Face-To-Face session, Barcelona, Spain
16	EURORDIS summer school, Barcelona

2.5. Organisations involved in EMA activities during 2016

There are currently 35 eligible patients' and consumers' organisations (Table 10). This list is published on the Agency [website](#) and includes links to their websites and a summary of their mission and objectives. This year two of the EMA eligible organisations were no longer eligible and one new organisation United Parent Project Muscular Dystrophy (UPPMD) joined the group

Any not-for-profit organisation that fulfils the following eligibility criteria is welcome to express its interest in getting involved in the work of EMA. These criteria include legitimacy, clear mission and objectives with an interest in medicines; representing patients or consumers throughout the EU and transparency. The current organisations include general umbrella organisations as well as those with emphasis in a specific area (such as rare diseases, HIV/AIDS, cancer etc.).

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](#)'.

Table 10: Eligible patients' and consumers' organisations working with the EMA

EMA eligible organisations	
1	AGE Platform Europe (AGE)
2	Alzheimer Europe (AE)
3	Debra International
4	European AIDS Treatment Group (EATG)
5	European Cancer Patient Coalition (ECPC)

EMA eligible organisations	
6	European Federation of Allergy and Airways Diseases Patients' Associations (EFA)
7	European Federation of Neurological Associations (EFNA)
8	European Foundation for the Care of Newborn Infants (EFCNI)
9	European Gaucher Alliance (EGA)
10	European Genetic Alliances' Network (EGAN)
11	European Haemophilia Consortium (EHC)
12	European Headache Alliance (EHA)
13	European Heart Network (EHN)
14	European Institute of Women's Health (EIWH)
15	European Liver Patient Association (ELPA)
16	European Multiple Sclerosis Platform (EMSP)
17	European Network of Fibromyalgia Associations (ENFA)
18	European Organisation for Rare Diseases (EURORDIS)
19	European Parkinson's Disease Association (EPDA)
20	European Patients' Forum (EPF)
21	European Prostate Cancer Coalition (EUomo)
22	European Public Health Alliance (EPHA)
23	Fabry International Network (FIN)
24	Global Alliance for Mental Illness Advocacy Networks (GAMIAN-Europe)
25	Health Action International (HAI)
26	International Alliance of Patients' Organizations (IAPO)
27	International Bureau of Epilepsy (IBE)
28	International Diabetes Federation European Region (IDF Europe)
29	International Patient Organisation for Primary Immunodeficiencies (IPOPI)
30	Myeloma Patients Europe (MPE)
31	Pain Alliance Europe (PAE)
32	Spinal Muscular Atrophy Europe (SMAE)
33	Thalassaemia International Federation (TIF)
34	The European Consumers' Organisation (BEUC)
35	United Parent Projects Muscular Dystrophy (UPPMD)

The EMA eligible organisations are the Agency's first port of call when a need arises to consult patients; however when the request is in a specific area not covered by the EMA eligible organisations, the Agency contacts other organisations for their expertise." In 2016, in addition to the 35 eligible organisations (Table 10), another 18 patients' and consumers' organisations also interacted with the Agency and are listed in Table 11.

Table 11: List of organisations consulted by EMA on specific areas

Organisations consulted by the EMA on specific areas	
1	Osteogenesis Imperfecta Federation Europe
2	Muscular dystrophy UK
3	Atypical haemolytic Uraemic Syndrome aHUS UK
4	ALD Life - adrenoleukodystrophy
5	EuropaDonna
6	Alpha-1 Foundation

Organisations consulted by the EMA on specific areas	
7	Dutch Association for People with Atopic Dermatitis (VMCE)
8	ALS Liga Amyotrophic lateral Sclerosis (Belgium)
9	Dravet syndrome Foundation
10	Swedish Breast Cancer Organisation
11	Cystic Fibrosis Europe
12	Lupus Europe, Lupus Italy
13	InSeNSU, patients with parathyroid gland function, Germany
14	Lymphoma Coalition, France
15	Tourettes action UK
16	Huntington's disease Association
17	L'Association PSP (Progressive supranuclear palsy) France
18	Migraine Trust, UK

2.6. Involvement in Networks and research projects

2.6.1. Elicitation of Patient Preferences and Values on Benefits and Risks project

Following experience gained in a small [pilot project](#) on patients' perspectives on benefits and risks of treatments, EMA, together with Myeloma UK and the University of Groningen, conducted a larger study involving 560 multiple myeloma patients from the UK. Through an online survey based on multi-criteria decision analysis, these patients were asked to express their willingness to trade-off between a product's favourable effects and its unfavourable effects. The study demonstrated that there is considerable diversity in how myeloma patients value the benefits and risk of treatments and this technique may complement other more direct methods for gathering patient views (e.g. face-to-face). More studies in other therapeutic areas are foreseen.

2.7. Exchange of practices of patient engagement

2.7.1. US Food and Drug Administration (FDA)

EMA and the FDA have set up a ['cluster'](#) on patient engagement, which is a new working group led by the Public Engagement Department, together with the Patient Representative Program within the FDA's Office of Health & Constituent Affairs. The cluster provides a forum for sharing experiences and best practices on how the two agencies involve patients in development, evaluation and post-authorisation activities related to medicines.

Both agencies consider the involvement of patients to be essential and areas of discussion will include the processes for selecting and preparing patients to take part in the agencies' activities, how to ensure that patients are independent and representative, and how to report on the impact of patient involvement. The first meeting of the cluster took place on 22 June by teleconference. Meetings are expected to occur three to four times per year and will be chaired jointly by FDA and EMA.

2.8. Next steps

In 2017, the Agency will continue to focus on the following areas:

- Continue with ongoing PCWP topic groups
- Implement recommendations of finalised topic groups
- Develop 2018-2019 work plans for working parties
- Continue to improve and expand training and support resources
- Continue with ongoing PCWP topic groups and implement recommendations of finalised topic groups
- Explore simplification of evaluation procedure of eligible organisations
- Further explore alternative methodologies for engagement, e.g. preference elicitation
- Establish principles for involvement of young people in EMA activities
- Continue to direct efforts towards expanded outreach

3. Interactions with healthcare professionals

3.1. Introduction

During 2016, the Agency continued to engage with healthcare professionals to facilitate the inclusion of a clinical practice perspective in EMA activities aimed at supporting medicines' development, evaluation and continuous improvement of the pharmacovigilance system.

The highlights of the year include the endorsement by the EMA's Management Board of a revised framework of interaction with healthcare professionals, the start of the second mandate of the Healthcare Professionals' Working Party (HCPWP) covering the period 2016-2019, and the establishment of an expert group with general practitioners/ family physicians.

In addition, efforts were directed to implement a longer-term planning for the HCPWP activities, aligned with the EU Medicines Agencies Network Strategy to 2020 and the EMA's multiannual working plan. The work developed by HCPWP topic groups also saw further progress with tangible outcomes in the fields of information for healthcare professionals, risk minimisation measures and social media.

Important steps were undertaken by the different healthcare professional organisations included in the EMA list of eligible organisations to ensure full compliance with transparency requirements. By the end of the year the list encompassed 30 organisations, including the European Respiratory Society which joined the list in 2016. As reflected in Figure 14, the representative organisations provide the basis of the EMA interaction with healthcare professionals (HCPs) and are the first port of call to identify individual experts and representatives to sustain the involvement of HCPs in the EMA work.

Throughout 2016, experts and representatives were involved in a number of specific activities related to benefit-risk assessment of medicines, throughout the entire medicine's lifecycle, as well as several core activities related with information on medicines and communication to healthcare professionals.

Figure 14: Regular interaction between the Agency and the network of European healthcare professional organisations

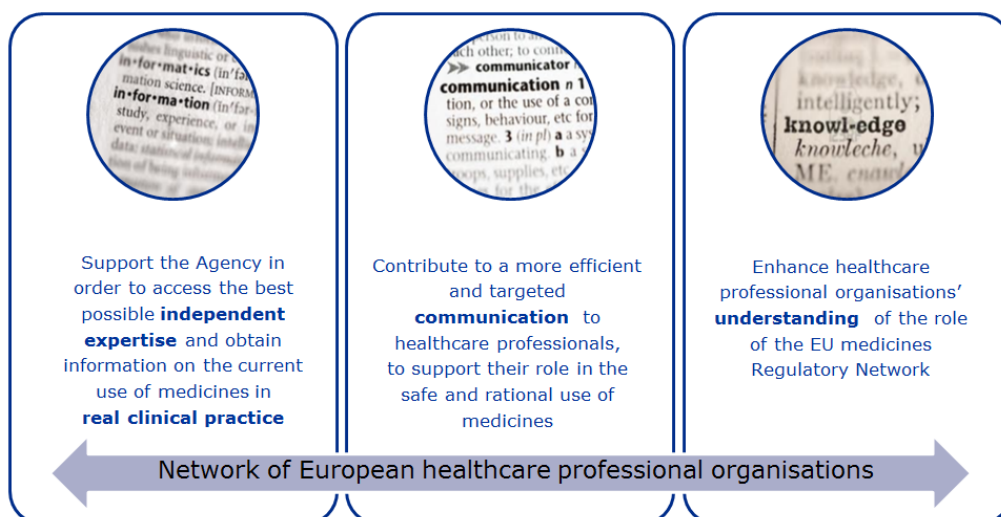
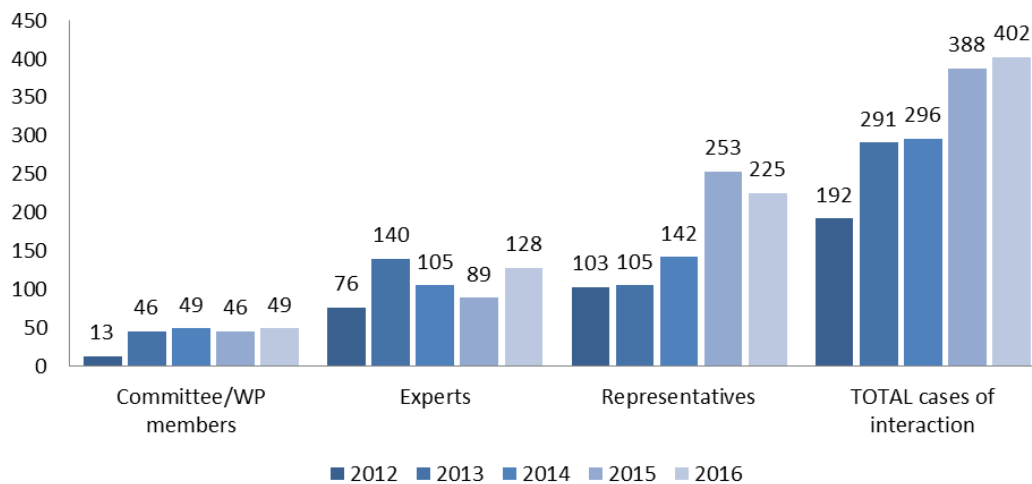


Figure 15 provides an overview of the sustained involvement of healthcare professionals in EMA core activities, which will be further elaborated on in the following sections.

Figure 15: Involvement of healthcare professionals as Committee/ Working Party members, experts and representatives of organisations

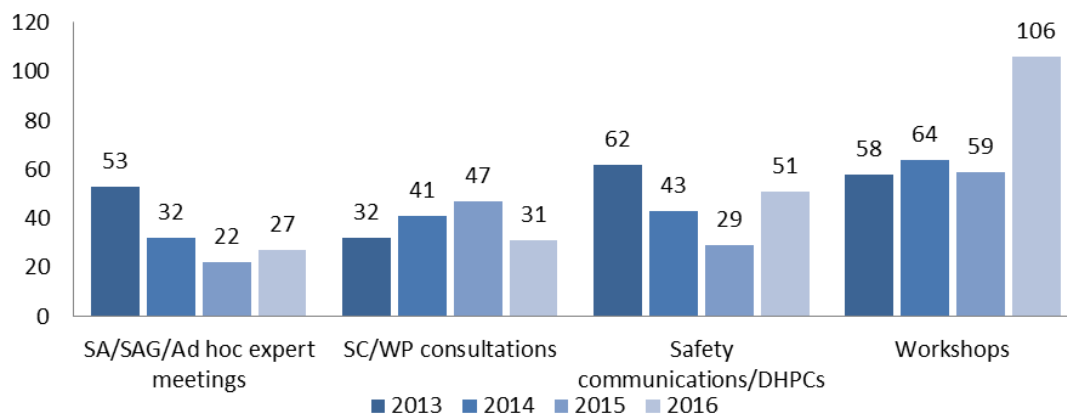


Although we continue to see an overall increase of cases of interaction over the years, it is worth noting that reported numbers are dependent on the activities that take place throughout the reporting years and fluctuations in numbers are likely due to the nature of the Agency's work.

In 2016, 10% of the total cases of interactions involved a general practitioner (an improved figure compared to the 4% recorded for 2015).

In Figure 16 the involvement of healthcare professionals in the Agency's scientific activities and workshops is illustrated.

Figure 16: Involvement of healthcare professionals in EMA activities (2013-2016)



As seen in Figure 16, the largest number of interactions occurred in the form of workshops. In fact 2016 was a workshop-rich year, including a dedicated workshop for general practitioners/family physicians. The year was also marked by increased participation in the review of safety communications and DHPCs. This is explained not only by the amount of communications but by the expansion in the number of reviewers, in order to include as much as possible, different healthcare professionals (physician, pharmacist, nurse). Variations on requests in 2016 for SAG/ ad-hoc expert meetings and scientific committee and working party consultations are explained by the number of requests linked to products undergoing assessment at the EMA.

3.2. Revised framework for interaction between EMA and healthcare professionals

In June 2016, the EMA Management Board adopted an overarching [stakeholder relations management framework](#) to structure stakeholder relations and better support strategic priorities, taking into account the general principles for stakeholder consultation outlined in the European Commission's Staff Working Document on [Better Regulation Guidelines](#).

A HCPWP topic group was asked to reflect on the need to review the framework for interaction between the Agency and healthcare professionals and it was agreed that the framework document should be updated to reflect the more proactive role of healthcare professionals in medicines development, evaluation and monitoring, as well as the principles for stakeholder consultation set out by the Better Regulation Guidelines. A [revised framework](#) was endorsed by the Management Board in December.

As part of the review, the framework was complemented with an action plan ([annex 1](#)) and a description of EMA activities where healthcare professionals are involved ([annex 2](#)).

3.3. Healthcare professionals in EMA activities and scope of representation

Healthcare professionals are involved in a wide array of Agency activities, either as representatives of healthcare professionals' organisations, representatives of their own organisations or as individual experts.

Figure 17 shows the different activities associated with these different types of representation.

Figure 17: Healthcare professionals in EMA activities and scope of representation



3.3.1. Healthcare professionals representing *healthcare professionals'* organisations

3.3.1.1. Membership in EMA management board and scientific committees

As described in Figure 17, healthcare professionals involved in the EMA Management Board and the Scientific Committees represent European healthcare professionals' organisations. These members are appointed by the European Commission in consultation with the European Parliament on the basis of their expertise. All members are required to have signed a Declaration of Interest and Confidentiality form in relation to their activities in the Agency.

Healthcare professionals are involved in governance activities via their membership in the Agency's

Management Board, where they have one representative.

At its June meeting, the European Medicine Agency's (EMA) Management Board welcomed new representatives of civil society who are joining the Board as members. Wolf-Dieter Ludwig, Head of the Department of Hematology, Oncology, Tumor Immunology and Palliative Care at Helios Klinikum Berlin-Buch, a hospital in Germany, has been reappointed to represent healthcare professionals' organisations.

In addition, healthcare professionals are represented in three of the six human scientific committees at the EMA (See Table 12). Activities performed by healthcare professionals in these committees include the assessment of paediatric investigation plans; the assessment of the quality, safety and efficacy of advanced-therapy medicinal products (ATMPs) and the assessment and monitoring of safety issues for medicines.

Table 12: Membership of healthcare professionals in EMA management board and scientific committees

EMA Management Board and Scientific Committees	Members / alternates
Governance:	
Management Board (MB)	2
Scientific Committees:	
Paediatric Committee (PDCO)	3 / 3
Committee for Advanced Therapies (CAT)	2 / 2
Pharmacovigilance and Risk Assessment Committee (PRAC)	1 / 1
TOTAL	14

3.3.2. Healthcare professionals representing *their* organisations

3.3.2.1. Membership of the Healthcare Professionals Working Party (HCPWP)

The Agency Human Scientific Committees' Working Party with Healthcare Professionals Organisations ([HCPWP](#)) was formally established in June 2013 to provide recommendations to the EMA and its Human Scientific Committees on all matters of direct or indirect interest to healthcare professionals in relation to medicines and to monitor the progress of interaction between the Agency and healthcare professionals. It is composed of representatives from 20 selected healthcare professionals' organisations that fulfil the [eligibility criteria](#) and representatives from the six Agency's human scientific committees as well as the Agency secretariat (Table 13). Additional observers include the European Commission and the Agency's Patients' and Consumers' Working Party (PCWP). The HCPWP is co-chaired by Gonzalo Calvo (EACPT) as a healthcare professional representative and Isabelle Moulon, on behalf of EMA. The HCPWP has observers from the PCWP who follow the work of this working party and present their particular perspective where necessary.

Table 13: Membership of working parties

Membership of working parties (WP)	Members / alternates or observers
HealthCare Professionals Working Party (HCPWP) + co-chair	19 + 1 / 17
Patients and Consumers Working Party (PCWP)	1
TOTAL	37

The HCPWP met three times in 2016. These meetings were mostly joint with the PCWP where a variety of topics were presented and discussed ranging from updates on different EMA core initiatives and projects to activities started by eligible organisations themselves. See section 1.7. for a comprehensive overview.

In addition to the joint meetings with the PCWP, the HCPWP dedicated its June meeting to discuss the revised framework of interaction with healthcare professionals and the framework of collaboration with academia (see section 3.3.2. for more details).

Figure 18: The HealthCare Professionals’ Working Party (HCPWP)



3.3.2.2. Topic groups of HCPWP

The topic groups were described in detail in the [annual report \(2015\)](#) and a report will be published with all recommendations and conclusions from the various topic groups. The aim of the topic groups is to enable brainstorming in smaller groups between plenary working parties’ meetings, promote further discussion on specific topics and allow better utilisation of time during the face-to-face working parties’ meetings. In Table 14, an update of the status of the existing topic groups is provided and in 2017 the working parties will discuss the potential launch of new topic groups.

Table 14: HCPWP topic group activities

HCPWP topic group activities	Status
Topic group on EMA-CHMP-PRAC projects on information on medicines	Finalised
Topic Group on Risk minimisation measures and assessment of their effectiveness	Ongoing
Topic group on Academia, learned societies and healthcare professional organisations	Ongoing
Topic group on Social Media	Ongoing

3.3.2.3. Workshops, meetings and consultations

This section includes additional interactions with healthcare professionals, which were not covered in section 1.8. A full overview of EMA workshops, conferences, ad hoc meetings and consultations involving healthcare professionals’ organisations can be found in Table 15.

Table 15: EMA activities involving healthcare professionals' organisations

Activities involving healthcare professionals' organisations	Number of representatives
Healthcare professional participation in workshops at EMA	106
Coordination meetings with HCP representatives of Scientific Committees	7
Scientific Committees/Working Parties consultations with HCPOs	5
HCPWP topic groups	49
Ad-hoc observers/experts attending HCPWP meetings	4
Observer at PCWP meetings	2
Comments to EMA draft guidelines, concept papers and reflection papers	2
European Hematology Association – teleconference on the Clinical Research Training in Haematology	2
European Academy of Neurology – teleconference on EMA session during annual congress	2
Review of e-learning module intended to support the users of the adrreports.eu portal	2
EMA consultation to (PCWP and) HCPWP on ADR website	1
Evaluation and re-evaluation of eligibility of organisations	31
EMA missions	8
TOTAL	225

Workshop with general practitioners

In the context of the implementation of the EMA framework of interaction with healthcare professionals' organisations, the Agency is striving to reinforce and promote the engagement with general practitioners (GPs).

In April 2016, a [dedicated workshop](#) was organised with the aim to reinforce and promote engagement with GPs and their representative organisations. Twenty representatives from three major organisations – the European Forum for Primary Care (EFPC), the European Union of General Practitioners (GPs) / Family physicians (UEMO) and the World Organization of Family Doctors (WONCA) Europe - attended the workshop.

In a welcome address by Isabelle Moulon she described the GPs as the 'missing link' in the Agency's interaction with healthcare professionals. This introduction was followed and elaborated by Guido Rasi (EMA Executive Director) who described the changing landscape of medicines. As the development of medicines and associated prices are changing, regulators also need to adapt to these changes; it is no longer sufficient to only evaluate the benefit-risk ratio, different ways of gathering evidence and the overall performance of a medicine in the 'real-world' setting are also required. He emphasised the importance of GPs in this change. In the context of their role of primary care, GPs often have the first contact with the patient and often accompany them throughout the life of their condition, which puts them in a unique position.

There is an increasing understanding that the generation of the evidence required after a medicine receives marketing authorisation needs to be planned from the beginning of its development and this is not possible without involving both the GP and the patient. Ivana Silva said the question is no longer about why GPs should be involved in regulatory activities but more specifically about how and when?

Presentations included those from EMA staff as well as from members of the GP representative organisations. A combination of formal presentations and breakout sessions were used to achieve an output of concrete actions in moving forward to working together. Some of the potential areas for collaboration include involvement in Scientific Advisory Groups (SAG), input in risk minimisation

measures both in terms of feasibility and impact on patients as well as document review and dissemination of information to their networks nationally and their patients locally.

The concrete actions to emerge from the meeting included the creation of a virtual expert group of general practitioners and the development of a joint position statement between EMA and the three organisations outlining concrete areas of collaboration as well as long-term recommendations. A full [report](#) is available on the EMA website.

Participation in written consultations addressing specific issues related with real world clinical practice

In line with the EMA framework for interaction with healthcare professionals, it is possible for a scientific committee, working party or drafting group to request additional input from relevant organisations on general clinical matters or on certain non-confidential aspects related with an on-going evaluation. The purpose of such consultations is to gather valuable input on certain aspects of clinical practice and standards of care that can support the scientific bodies on its further discussions related with on-going evaluations or guideline development. Table 16 lists the consultation carried out in 2016.

Table 16: Committee/Working party consultations with healthcare professional organisations

Committee/ working party	Subject	Contribution of healthcare professionals
CHMP	CHMP consultation on antipsychotic medicine used in adults and children for a number of mental and other brain disorders	<p>The consultation took place in the context of a referral procedure to harmonise the marketing authorisations for a specific product and associated names in the EU.</p> <p>The questions to the healthcare professionals' organisations (HCPOs) mainly pertained to the clinical value of the indications (section 4.1) and dosing recommendations in clinical practice (section 4.2), as well as the contraindication for the medicine due to central nervous system depression, and whether it was possible to define the severity/degree of central nervous system depression due to alcohol or other depressant medicinal products, and whether there were specific cases where the use of the medicine should be contraindicated.</p> <p>Based on the review of all available data, the consultations with the HCPOs and Scientific Advisory Group (SAG) Psychiatry, the CHMP recommended revisions to harmonise the product information.</p>

3.3.3. Healthcare professionals as individual experts

When healthcare professionals are involved in EMA activities on product-specific issues, they do so as individual experts. The Agency asks relevant healthcare professional organisations to identify experts who, on the basis of their individual clinical experience, and subject to the assessment of declared interests and signed confidentiality agreement, can provide their valued input. A list of these activities is shown in Table 17.

Table 17: EMA activities involving healthcare professionals as individual experts

Activities involving individual experts	Number of Experts
Review of documents	
Safety communications	41
DHPCs	10

Activities involving individual experts	Number of Experts
Involvement in medicines evaluation	
Participation in SA/SAGs/Ad hoc expert group meetings	27
Committee consultations	
CHMP consultation on labelling and package leaflet of emergency contraceptives	10
PRAC consultation on product related educational material	2
QRD/PRAC written consultation on risk minimisation of medication errors	7
Involvement in surveys/questionnaires and user testing	
Risk minimisation measures and assessment of their effectiveness (questionnaires for 4 case studies)	15
User testing of clinical data publication website prototype (phase 1 and 2)	8
Other consultations	
Consultation on storage conditions for vaccines	6
Review of ADR e-learning module, as part of the EudraVigilance training curriculum	2
TOTAL	128

3.3.3.1. Healthcare professional involvement in scientific meetings

As described in Section 2.2.3.2.2. Scientific Advisory Groups (SAGs) are convened by the CHMP or the PRAC to provide advice in connection with the evaluation of specific types of medicines or treatments.

Experts are involved in SAG/ ad-hoc meetings in order to support scientific discussions related with the evaluation of new marketing authorisation applications and changes in indications of already approved medicines. Through the network of diverse European healthcare professional organisations, the Agency called upon 27 individual experts to participate in SA/SAG/ad –hoc expert group meetings and bring additional expertise on clinical practice in specific domains during 2016. This expertise was provided on a variety of therapeutic areas and medical fields, including clinical immunology, endocrinology, ophthalmology, HIV, infectious diseases, oncology, haematology, radiology, neurology and paediatrics.

EMA Geriatric Expert Group (GEG)

The Agency's Geriatric Expert Group (GEG) provides scientific advice to the CHMP and the European Medicines Agency secretariat on issues related to older adults. Its work includes:

- giving input related to geriatrics on guidelines under consultation and developing guidelines at CHMP request: in 2016 the frailty Points to Consider were released for public consultation;
- giving advice on geriatric aspects of the development, assessment or safety monitoring of medicines: in 2016 it provided input in the CHMP geriatric pilot on new MAAs;
- taking part in meetings where expertise on geriatrics is needed;
- contributing to the geriatric implementation plan.

The majority of the members of the Geriatric Expert Group (GEG) are practising healthcare professionals. In 2016 they have been consulted for input regarding the drafting of guidelines, the provision of Scientific Advice and support to the PRAC in referrals pertaining to the older population.

3.3.3.2. Participation in written consultations

The purpose of this type of consultation is to gain a better understanding of whether specific elements

of the product information and package design (e.g. labelling; expression of strength; posology recommendations; instructions for use; colour differentiation strategy) are sufficiently clear. Furthermore there is a focus on whether additional risk minimisations measures (e.g. key messages to include in educational materials) can reduce potential risk of medication errors in the context of clinical practice reality and facilitate the appropriate and safe use of the medicinal product under assessment. See Table 15 for consultations carried out throughout 2016.

Table 18: Committee/Working party consultations in writing with healthcare professional (individual experts)

Committee/ WP	Medicinal product	Contribution of healthcare professionals
CHMP	Emergency hormonal contraceptives	In the context of a referral procedure to address the effects of interaction of a specific product and associated names with certain enzyme inducers to treat HIV, epilepsy, tuberculosis or with preparations containing St John's wort, healthcare professionals were consulted on the proposed wording to be included on the outer carton and in the package leaflet to ensure this was as clear and understandable as possible. The responses highlighted a low level of awareness of this type of interactions, underlining the need for proactive national communications on the outcome of the present review. For that reason the CHMP discussed the key elements for communication to healthcare professionals and patients to facilitate the communication at national level.
PRAC	Anti-cancer medicine indicated for the treatment of advanced melanoma in adults	In the context of a periodic safety update covering the review of the risk minimisation measures (RMMs) put in place for the medicine at the time of granting of the marketing authorisation, healthcare professionals were consulted on the usefulness of educational materials to ensure they are informed of the key immune-related adverse reactions (irARs). Their input was taken into account in the PRAC assessment to maintain the Healthcare Professional Brochure as an important tool to minimise risks of irARs with this medicine, together with the Patient Information Brochure (including Alert Card).
EMA	Vaccines	Healthcare professionals, including general practitioners and pharmacists, were consulted in order to gather additional elements to inform a discussion on the provision of data-driven information in the SmPC, regarding acceptable deviations from standard storage conditions for vaccines.
QRD/PRAC	Anti-diabetic medicine	Due to the amount of current/future medicines containing same active substances and/or fixed-dose combinations for different indications, healthcare professionals were consulted on what could be improved to minimise the risks of medication errors to ensure the safe use of these medicines. Input was taken into account at the time of the review of the draft product information provided at D121 of the procedure (SmPC, labelling and package leaflet) and of the draft mock-ups of the labelling (outer carton and labels).

3.3.3.3. Review of EMA information

The EMA is responsible for providing information about medicines authorised via the centralised procedure, which includes information directed to stakeholders. During the preparation of this information, the Agency interacts with healthcare professionals' organisations to ensure that the communication is adequately formulated and comprehensible to the target audience.

Throughout 2016, healthcare professionals were asked to provide their views on several types of

documents:

- The **Summary of Product Characteristics** (SmPC) is a key part of the marketing authorisation of all medicines authorised in the European Union and the basis of information for healthcare professionals on how to use a medicine safely and effectively.
- **Safety communications** refer to documents that are specifically addressed to the public, patients and healthcare professionals on authorised medicinal products and that convey an important (emerging) message relating to the product (e.g. a product is withdrawn or suspended for safety reasons, has a new contraindication or warning, or there is a product defect). In addition are safety communication written when additional measures have been included in a medicine's risk management plan to reduce the risk of medication errors.
- **Direct healthcare professional communications** (DHPCs) are usually disseminated by one or a group of marketing authorisation holders for the respective medicinal product(s) or active substance(s), either at the request of a national competent authority or the Agency, or on the marketing authorisation holder's own initiative.
- The **shortages catalogue** contains information on medicine shortages that affect or are likely to affect more than one European Union (EU) Member State, where the European Medicines Agency has assessed the shortage and it provides recommendations to patients and healthcare professionals across the EU;

Risk communication

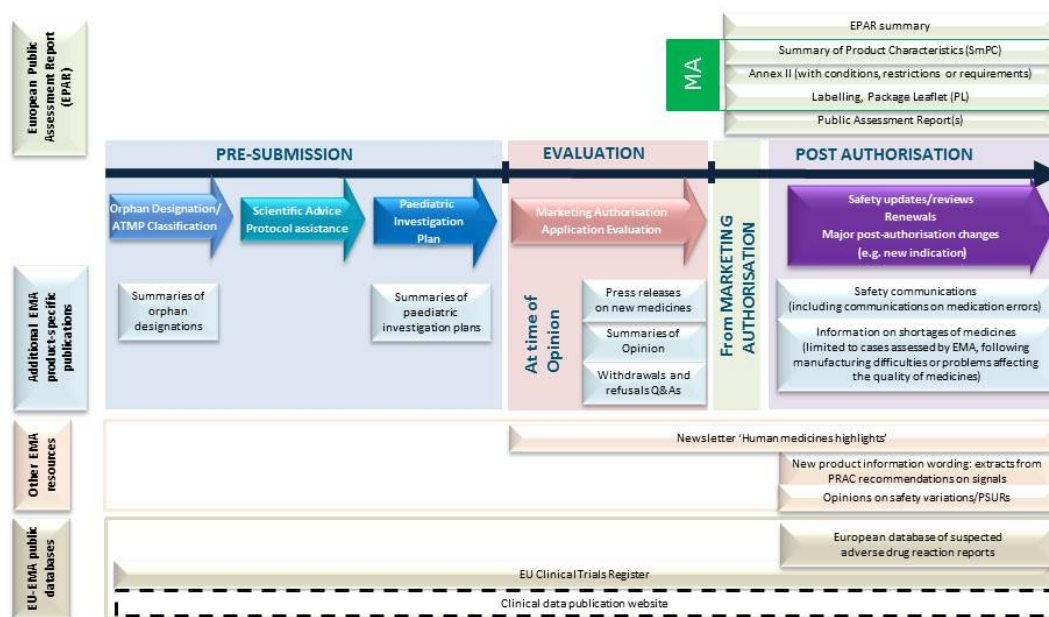
A main focus of the Agency's communication policy is to inform stakeholders of key safety information that the Agency produces. EMA public information on 'start of safety referrals' as well as 'summary of recommendations' are written specifically with the intention to target patients and healthcare professionals, and the Agency's policy is to disseminate these communications at the time of their publication to the key EU organisations in the field. In order to promote clarity of the messages prepared, the Agency also seeks specific input from relevant reviewers in the target groups during the drafting process. The same applies to direct healthcare professionals' communications (DHPCs).

In 2016, a total of 22 experts nominated by healthcare professional organisations (HCPOs) with different specialities and clinical backgrounds were involved in the review of 19 safety communications and 7 DHPCs. Most of the feedback received was positive with pertinent suggestions used to reinforce the clarity of the messages to be conveyed.

Mapping EMA sources of information to healthcare professionals

The infographic (Figure 19) was developed as part of the work undertaken by the HCPWP topic group on information for healthcare professionals in 2016. The objective was to provide additional insight to the different sources of information that are available on the EMA website.

Figure 19: EMA sources of information for healthcare professionals, patients and the general public



3.3.4. Interactions with Academia

As described in the annual report (2015), an internal survey was conducted to collect information on the current interactions between the Agency and academia. In addition, healthcare professionals and learned societies were consulted in the context of the HCPWP meeting in June and informal exchanges were organised with representatives of several European research infrastructures (see Table 19) in the biomedical field as well as with other academic organisations.

Table 19: List of organisations consulted on the collaboration with academia

Academic organisation	
1	European Infrastructure for Translational Medicine (EATRIS)
2	European Clinical Research Infrastructure Network (ECRIN)
3	Biobanking and Biomolecular Resources Research Infrastructure (BBMRI)
4	European Research Infrastructure for Imaging Technologies in Biological and Biomedical Sciences (EU-BioImaging)
5	European Infrastructure for Phenotyping and Archiving of Model Mammalian Genomes (INFRAFRONTIER)
6	Infrastructure for Systems Biology Europe (ISBE)
7	European Infrastructure for Chemical Biology (EU-OPENSREEN)
6	EU Infrastructure for life-science information (ELIXIR)
9	European Organisation for Research and Treatment of Cancer (EORTC)
10	European Association for Communication in Healthcare (EACH)

In parallel, a survey was launched to consult with the academic world on the following objectives:

- Assess the degree of awareness among academics of the role of regulators and of the existing activities and incentives supporting medicine development;
- Refine regulators' understanding of academia's needs;
- Identify opportunities for a greater collaboration in order to better support academia in

generating new medicines that meet regulatory standards.

The survey, run from February to April 2016, was very successful in terms of response and richness of input. A total of 1016 responses were received and 877 responses were considered valid (respondents completed at least the basic profiling questions; double entries and for-profit affiliated respondents were cleared).

The results indicated the need for education and training to enhance awareness of the role and activities of regulators, which could also act as a means to increase academia's engagement in regulatory science activities and research. Survey respondents strongly agreed on the need for increased regulatory support to help them translate academic research into novel methodologies and medicinal products. Finally, there was a clear request to strengthen communication and knowledge exchange opportunities. This should ensure that the best scientific expertise and academic research continue to be available to support decision making in regulatory processes and that academia is offered a robust, multi-stakeholder platform for dialogue at EU level (see [survey report](#) for more information).

The priorities identified via the survey have been captured in the drafting of the framework and in the list of actions to be implemented once the framework will be formally adopted.

Healthcare Professionals Working Party (HCPWP) workshop with academia

During its June 2016 meeting, the Healthcare Professionals' Working Party (HCPWP) has hosted a [workshop](#) focused on the development of a framework of collaboration between EMA and academia (see also [workshop report](#)).

In his opening statement Guido Rasi, EMA executive director, captured the rationale of this endeavour: "EMA wants to move to a new level of collaboration with academia. Science is progressing fast and we see an unprecedented level of complexity in the development and evaluation of new medicines. Academia play an important role in helping the EU medicines regulatory network to keep abreast of the opportunities and challenges brought by science and to have access to the right expertise to evaluate these innovative medicines. Interaction with EU regulators and a better understanding of the regulatory environment can help academia translate their discoveries into patient-focused medicines. I believe that working more closely together will bring great benefits to public health".

Proposal for a framework of collaboration

The overall aim of the framework is to reinforce and further develop the collaboration between the Agency and academia by clarifying scope, formalising and structuring interactions in the wider context of the European medicines regulatory network. The framework will cover areas of common interest for the EMA and academia, clearly stating that specific queries relating to a specific product and/or regulatory procedure will fall outside its scope.

It should be noted that although the draft framework of collaboration with academia and the framework of interaction with healthcare professionals share common objectives, the first will focus primarily on research and education, whilst the latter will continue to focus on clinical practice.

The endeavour of defining a coherent framework of collaboration with academia was supported by great interest and commitment from all stakeholders involved. The importance of striking the right balance between the ambitions, the challenges, the existing assets and the feasibility of a list of actions account for the substantial amount of ponderation and time taken to shape the framework. The process of refinement will continue and 2017 should see the coronation of all efforts with the delivery of a framework of collaboration adopted by the Management Board of the Agency.

3.4. EMA awareness-raising activities

In order to build capacities and promote further awareness on how the Agency is involving healthcare professionals in its activities, the Agency engages in various activities. The Resource page contains useful links to relevant workshops as well as the 'videos' and related documents or pdf versions of the slides with text (Section 1.9.4.).

In addition, EMA staff have participated in several specific meetings and conferences organised by healthcare professionals' organisations in 2016 as shown in Table 20.

Table 20: EMA participation in external healthcare professionals' meetings

Organiser/Event	
1	European Association of Hospital Pharmacists (EAHP) 21st congress, Vienna
2	European Congress of Endocrinology (ESE), Munich
3	European Hematology Association (EHA) clinical research training in hematology Workshop, Milan
4	European Forum for Primary Care (EFPC) conference, Riga
5	European Association for the Study of Diabetes (EASD) conference, Munich
6	European Committee for Treatment and Research in Multiple Sclerosis (ECTRIMS) – EMA – European Multiple Sclerosis Platform (EMSP), London
7	Italian Federation: Annual meeting of primary care, Cagliari
8	United European Gastroenterology (UEG) conference, Vienna
Meetings organised in collaborations with EMA	
	'Regulatory symposium' during the 12th European Congress on Epileptology organised by the International League against Epilepsy (ILAE), Prague
	'Regulatory symposium' during the Annual Congress of the European College of Neuropsychopharmacology (ECNP), Vienna

3.5. Organisations involved in EMA activities in 2016

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 healthcare professionals' organisations involved in the European Medicines Agency activities](#)'.

A new organisation was added to the list of EMA eligible organisations in 2016 – the European Respiratory Society. The 30 healthcare professionals' organisations are shown in Table 21 and are also published on the Agency [website](#), including links to their websites and a summary of their mission and objectives.

Any not-for-profit organisation that fulfils the following eligibility criteria is welcome to express its interest in getting involved in the work of EMA. These criteria include legitimacy, clear mission and objectives with an interest in medicines; representing patients or consumers throughout the EU and transparency. The current organisations include general umbrella organisations as well as those with emphasis in a specific area (such as rare diseases, HIV/AIDS, cancer etc.).

Table 21: Eligible healthcare professionals' organisations working with the EMA

Name of Organisation	
1	European Academy of Allergy and Clinical Immunology (EAACI)
2	European Academy of Paediatrics (EAP)
3	European Academy of Neurology (EAN)

Name of Organisation	
4	European AIDS Clinical Society (EACS)
5	European Association for Clinical Pharmacology and Therapeutics (EACPT)
6	European Association of Hospital Pharmacists (EAHP)
7	European Association for the Study of Diabetes (EASD)
8	European Association of Urology (EAU)
9	European College of Neuropsychopharmacology (ECNP)
10	European Federation of Internal Medicine (EFIM)
11	European Forum for Primary Care (EFPC)
12	European Haematology Association (EHA)
13	European League Against Rheumatism (EULAR)
14	European Renal Best Practice (ERBP)
15	European Respiratory Society (ERS)
16	European Society of Cardiology (ESC)
17	European Society of Endocrinology (ESE)
18	European Society for Medical Oncology (ESMO)
19	European Specialist Nurses Organisations (ESNO)
20	European Society of Oncology Pharmacy (ESOP)
21	European Stroke Organisation (ESO)
22	European Society of Radiology (ESR)
23	European Union of General Practitioners / Family physicians (UEMO)
24	European Union Geriatric Medicine Society (EUGMS)
25	European Working Group on Gaucher Disease (EWGGD)
26	Health Care Without Harm Europe (HCWH Europe)
27	International League Against Epilepsy (ILAE)
28	Pharmaceutical Group of the European Union (PGEU)
29	Standing Committee of European Doctors (CPME)
30	United European Gastroenterology (UEG)

Occasionally, the Agency needs to approach organisations that have not undergone the voluntary process of applying for eligibility due to the need to consult on a specific area not covered by the eligible organisations. These organisations, which provided experts for Scientific Advice; Scientific Advisory Group meetings; contributed to HCPOs consultations; and whose representatives participated in workshops/conferences, are listed in Table 18 below.

Table 22: List of organisations consulted by EMA on specific areas

Name of Organisation	
1	European Board & College of Obstetrics and Gynaecology (EBCOG)
2	European Committee for Treatment and Research in Multiple Sclerosis (ECTRIMS)
3	European Organisation for Research and Treatment of Cancer (EORTC)
4	European Paediatric Neurology Society (EPNS)
5	European Psychiatric Association (EPA)
6	European Renal Association – European Disability and Transplant Association (ERA-EDTA)
7	European Society for Paediatric Gastroenterology, Hepatology and Nutrition (ESPGHAN)
8	European Society of Clinical Microbiology and Infectious Diseases (ESCMID)
9	European Society of Ophthalmology (SOE)
10	Society for Ophthalmo-immunoinfectivology in Europe (SOIE)

3.6. Next steps

In 2017, the Agency will continue to focus on the following areas:

- Continue with ongoing HCPWP topic groups
- Implement recommendations of finalised topic groups
- Develop 2018-2019 work plans for working parties
- Continue to reinforce and promote engagement with general practitioners, in close collaboration with their representative European organisations
- Structure contributions from experts in medication errors
- Expand participation of specialist nurses in EMA activities
- Explore simplification of evaluation procedure of eligible organisations
- Continue to direct efforts towards expanded outreach

Conclusions

2016 was another successful year of collaboration with stakeholders. Using the consulting tools described in section 1 and the extensive engagement methodologies developed over two decades of existence; best practices are now well established for use when planning and co-ordinating interactions with stakeholders. As can be seen from the pages in this report, the involvement of patients, consumers and healthcare professionals is extensive and in every aspect of medicines evaluation, workshops and document review in order to enable a more proactive approach in line with the Agency's priorities.

From the well-established working parties to fostering new links, such as those already initiated with general practitioners, academia and young people, continued outreach and maintenance of existing relationships is essential to the work of EMA and to ensuring trust and confidence in regulatory procedures.

Objectives for the year ahead include reinforcement of EMA's outreach to primary care physicians (GPs) and specialist nurses, finalisation of a framework for collaboration with academia and implementation of the action plan as part of the European Medicine Regulatory Network and further establishing the principles of involving young people in EMA activities.

In addition to these new activities, the objectives for 2017 include managing and streamlining the work of the two working parties (PCWP and HCPWP) including implementing current work plans and looking ahead to develop work plans for the next two years (2018-2019). The success of several topic groups has resulted in recommendations that will be implemented and published; the groups that have achieved their objectives will be on 'standby' for future reactivation, as needed. New groups will be created for future work that aligns with the EMA work programme and the EU Network Strategy to 2020.

While the opportunity for a public hearing did not present itself during 2016, EMA continues to work closely with and to support the PRAC in the context of safety referrals to find the optimal engagement method to ensure the input of concerned patients, consumers and healthcare professionals. Following a 'mock' public hearing, EMA is ready to conduct its first public hearing, likely in the course of 2017.

A survey of the key stakeholder groups of the Agency will be conducted to ensure communication and stakeholder engagement activities are effective and continue to meet expectations. Actions will be implemented to address the feedback received.

The Public Engagement department plays a role in supporting the European Medicines Agency achieve its mission through its interactions with stakeholders who, prescribe, supply and use medicines.

As always, this work would not be possible without the collaboration and commitment of our eligible organisations, working parties and experts who contribute to enhance the regulation of medicines in Europe.

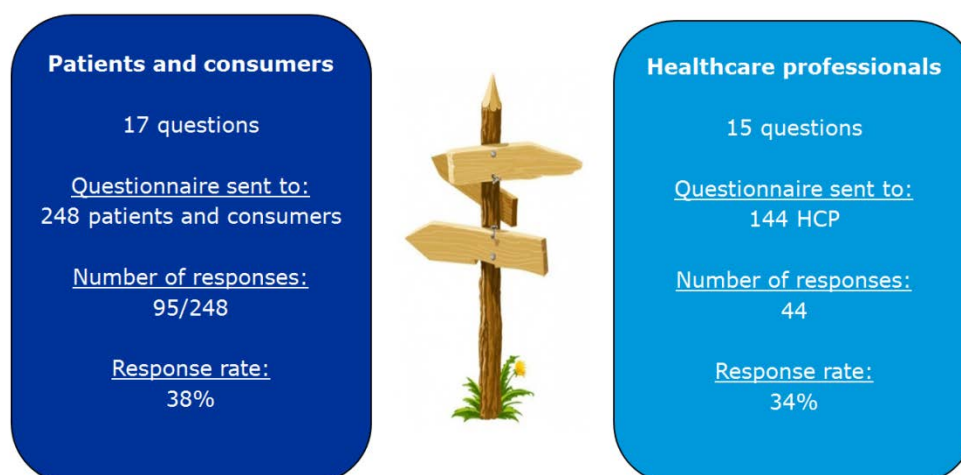
Annex I.: Satisfaction survey

Introduction and methodology

The satisfaction survey is conducted every two years as per the request of the EMA Management Board. The main aim of the survey is to gather feedback from patients, consumers and healthcare professional on EMA activities where they have been involved, to provide reflection on stakeholder involvement, understand the level of satisfaction and identify areas where further improvement is required.

For the purpose of the survey, two surveys were prepared, one for patients and consumers and the other for healthcare professionals. In Figure 20, we can see the summary information about both satisfaction surveys. There were 17 questions in total for patients and consumers and the survey was disseminated to everybody who had interacted with EMA over the course of 2016. A response rate of 38% was obtained with 95 of the 248 patients and consumers responding to the survey. Similarly the survey for healthcare professionals had a total of 15 questions and was shared with 144 individual healthcare professionals who had interacted with EMA in 2016. A total of 44 responses were obtained giving a response rate of 34%.

Figure 20: Summary for patients and consumers, and healthcare professionals



Results

General information

Both surveys were grouped into five sections; general information, general interactions, logistics, future participation as well as a section for any other comments. All questions were mandatory and while the format was based on a rating scale, there was the possibility to include free text for each response.

Figure 21 and Figure 22 provide demographic information on the respondents. The majority of patient and consumer respondents were from the United Kingdom, followed by Belgium, Germany and Netherlands. A different spread of nationalities is observed in the healthcare professionals with the majority being from Italy, followed by the Netherlands, Belgium and Spain. In both cases, the majority of respondents had been involved with the EMA between 1-3 years.

It is important to remember that these demographics do not reflect all patients, consumers and healthcare professionals involved in EMA activities but only those who responded to the survey.

Figure 21: Demographics of patients and consumers

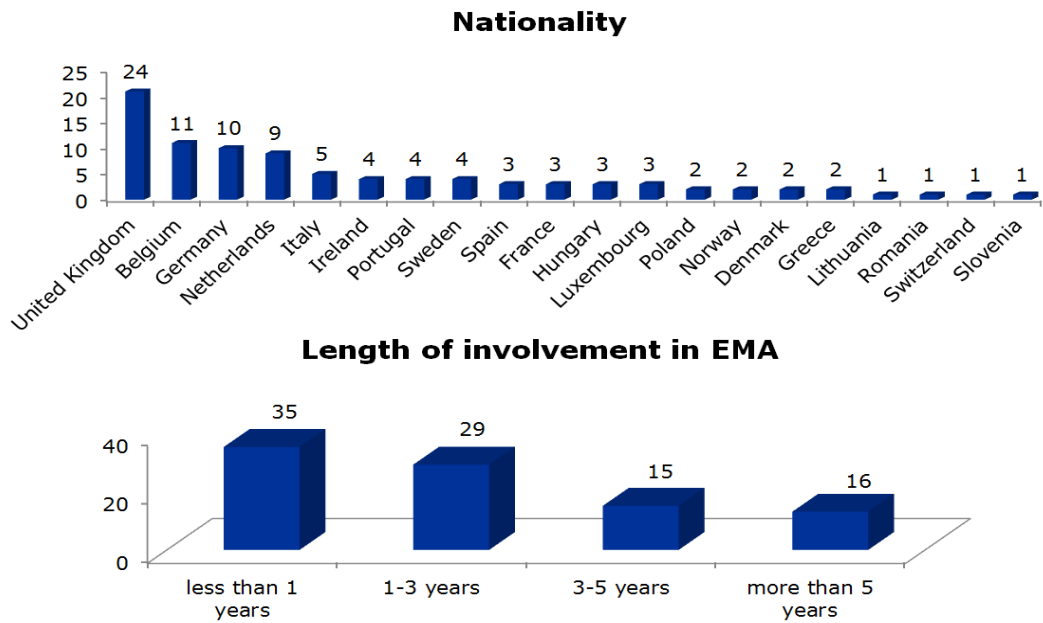
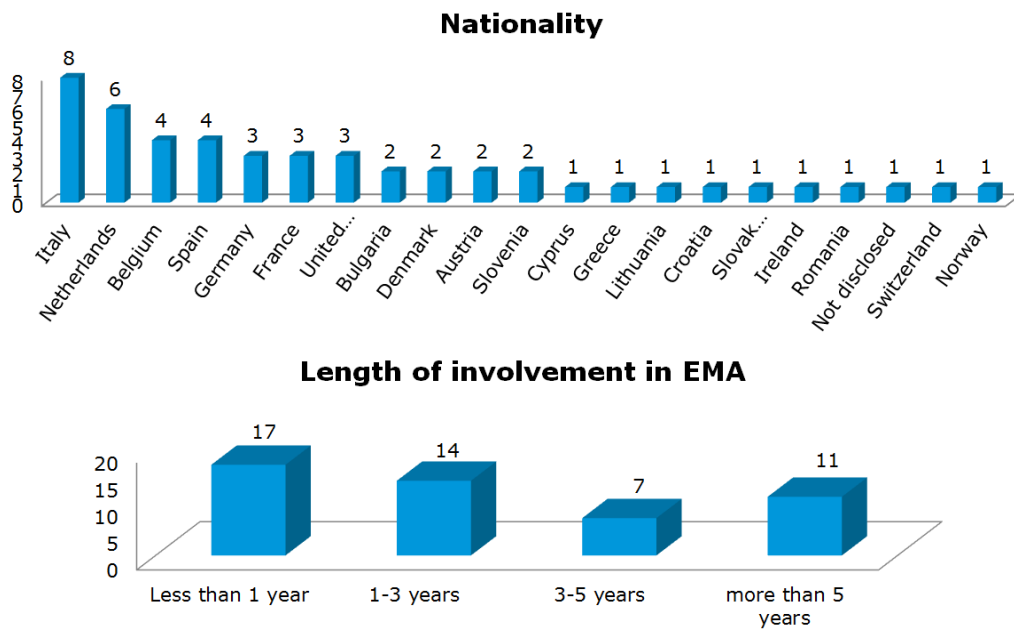


Figure 22: Demographics of healthcare professionals



The survey also asked respondents to indicate in which activities they had been involved in 2016 and the wide variety of categories are shown in Figure 23 for patients and consumers and Figure 24 for healthcare professionals. Membership of Healthcare Professionals Working Party (HCPWP) or Patients and Consumers Working Party (PCWP) was one of the main activities, followed by participation in workshops and expert meetings in medicines evaluation

Figure 23: Activities where patients and consumers participated

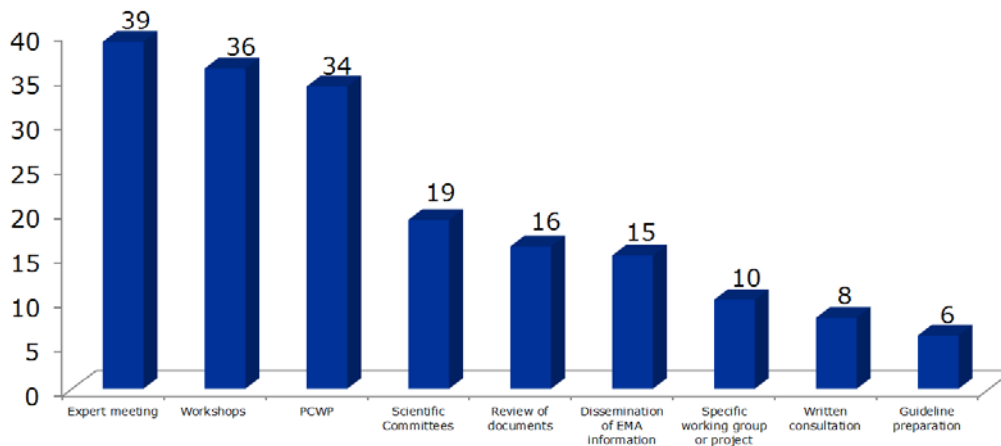
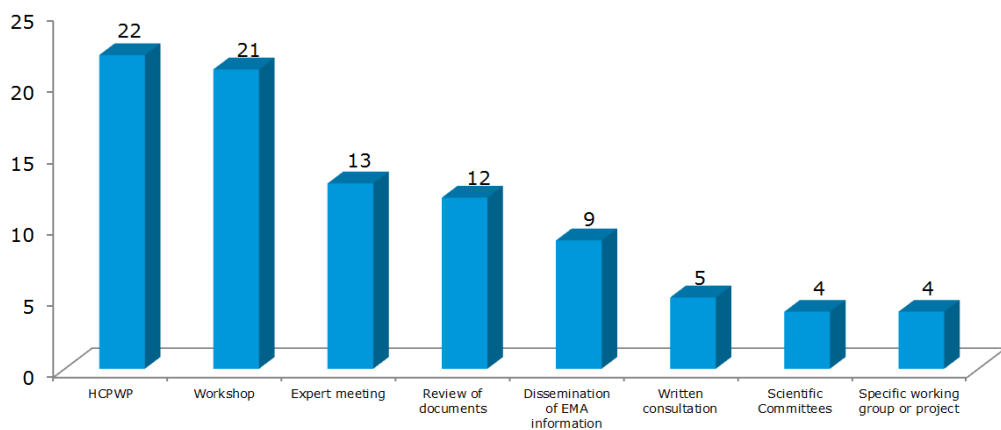


Figure 24: Overview of activities, in which healthcare professionals participated

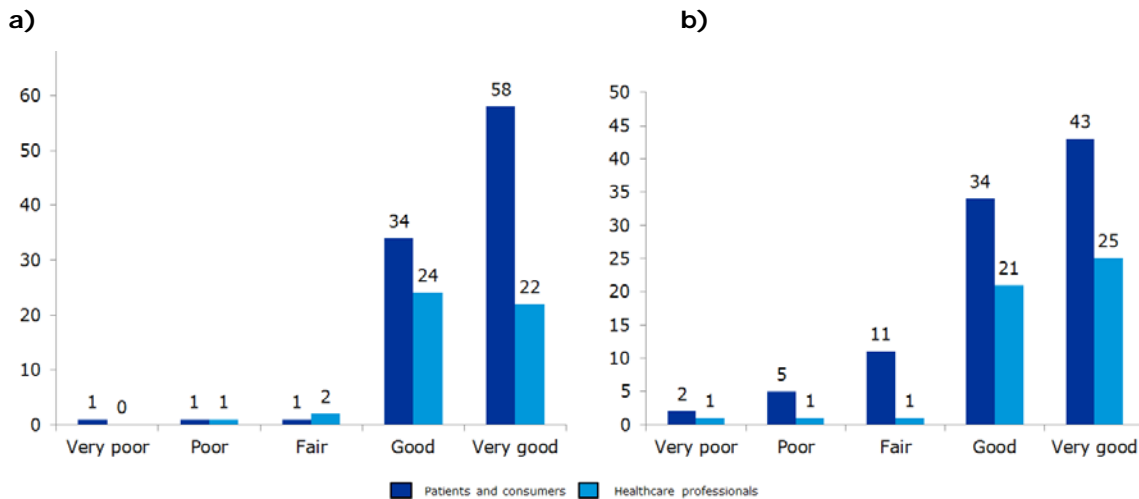


General interactions

The majority of all respondents indicated a high satisfaction (good to very good) for the categories a) interactions with EMA and b) the level of support received prior to a specific activity. However, several (18) patients and consumers rated the support received as fair to very poor and comments on these questions highlighted issues with the registration process and in some instances, there was insufficient support provided prior to involvement in an activity. It is important to remember that the satisfaction surveys cover all activities where patients are involved and also include logistics.

Healthcare professionals' comments reflected a perception of EMA as responding rapidly to questions and as being constructive with their responses. Several respondents highlighted the personal approach and politeness of staff. However, they commented on delays in receiving necessary documents which impacts on ability to prepare when participation in a meeting; this aspect needs to be improved.

Figure 25: Overall interaction and level of support prior to the meeting



Comments from patients and consumers

+ "People at EMA were **very quick to react** and help me, very good feeling."
 "Members and managers of the scientific team have been very helpful to phone me and to **explain the procedures.**"
 "Helpful, friendly, people in EMA are great! It's my **pleasure working with them.**"

- "... **registration online** is too heavy takes too long."
 "The **credit card system for food**, the unbelievably complex system to reclaim expenses or secure a hotel room all seem designed to stop people from claiming money back."
 "I did not receive any **support prior to the meeting** other than logistics/admin"

Comments from healthcare professionals

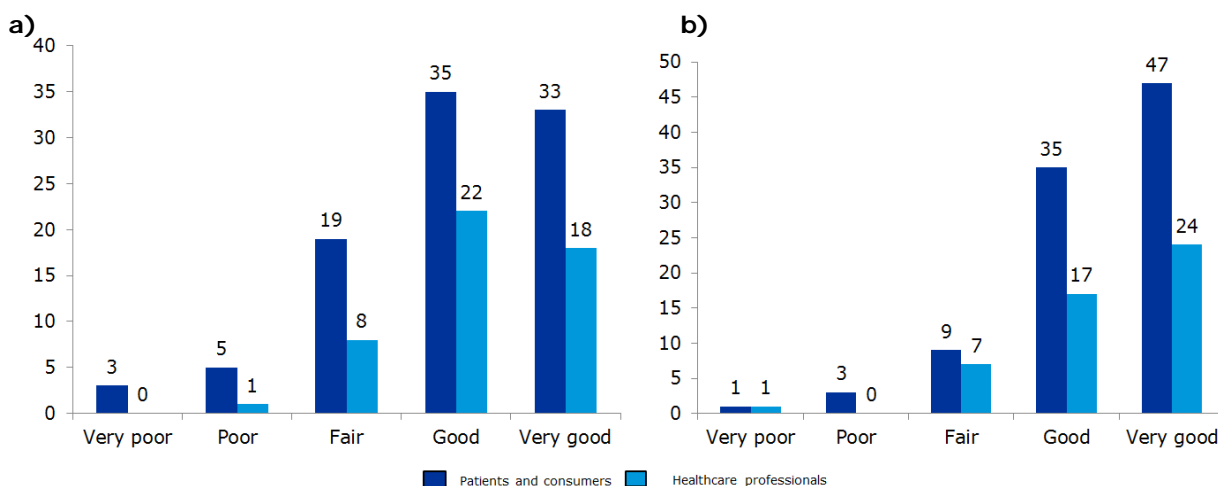
+ "The experience is that **the office is very swift and constructive** in responding and to my satisfaction."
 "This is very effective but I also reward **the personal approach** very much, it is an example of showing interest from both sides."
 "Very fast and **very polite** and efficient."

- "One issue may be **timing** a little. Generally there is considerable paperwork and not so much time to appropriate address everything."
 "I **don't get involved in preparations preceding the Scientific committee meetings** and therefore can't provide input. Trying to give input during the meeting is very difficult when you don't have had access to documents."

Stakeholders were also requested to rate both a) the level of follow-up received and b) the EMA initiative of involving stakeholders (Figure 26). While the majority rated follow up as good to very good, more than 25 patients and consumers felt that follow-up could be improved. Healthcare professionals provided positive comments, with particular attention to willingness of EMA to gain clinical practice insights and to further involve general practitioners in the work of EMA. Once again, it

was mentioned that final documents are often delivered too late.

Figure 26: Follow-up after participation and stakeholder involvement initiative



Comments from patients and consumers

+

"I always get the final drafts, so that's okay."

*"I felt that **patients opinions and information are taken seriously for further decisions**. This is for us very important and I feel grateful that I could take part in this process for the new drug."*

*"Great that it is happening at all, and beyond that, the fact that it seems **a well-integrated process within EMA**, and very supportive of people becoming involved."*

-

*"I had a couple of emails thanking me for participation, and a promise that I would **receive the final outcome of the meeting** as well - so far that **hasn't happened**."*

"I got a thank you. That is all."

*"It's very hard to **gain information about drug assessment progress** either by telephone or on your very confusing website."*

Comments from healthcare professionals

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*"Very good because of the double service, **receiving information by mail and online**."*

*"Glad to see that further involvement **of general practitioners/family doctors** is being facilitated."*

*"Extremely well and **experience an open mind and willingness** to view issue from our scope of practice."*

-

*"**Final documents** incorporating suggestions **not made available**."*

*"I think **involving HCPs is difficult** in an environment that has to be very 'regulatory', i.e. has a main focus on updating SPCs and writing HCP communications. And that can't quite understand that HCPs rarely read SmPCs."*

Logistics

In Figure 27 and Figure 28, we see how patients, consumers, and healthcare professionals rated a) the practical arrangements and b) the organisation of the meetings and financial support related to involvement in various activities. We can see high level of satisfaction with practical arrangements and meeting organisation. Some stakeholders appreciated the smooth process of involvement, the amount

of guidance they received and navigation throughout the meeting. Survey participants addressed the issue of financial support. In many cases the level of financial support is not considered adequate. It was expressed that the experts are not reimbursed for the time spent in EMA.

Figure 27: Practical arrangements and meeting organisation

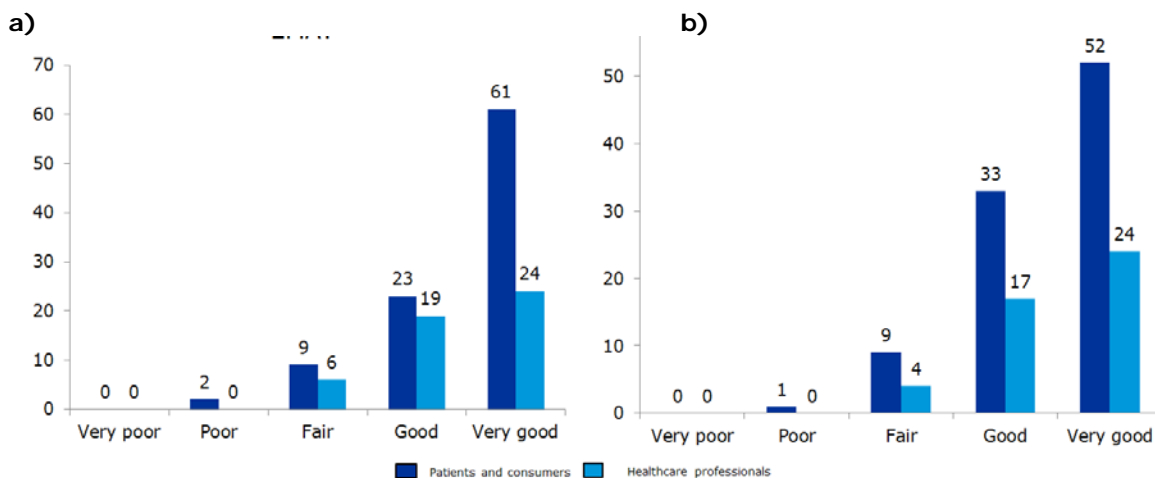
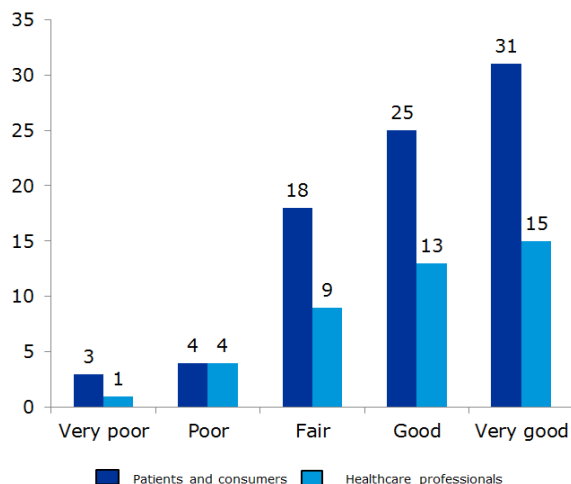


Figure 28: Overall level of financial support provided



Comments from patients and consumers

"It's always perfect. I admire all the efforts of people involved in such arrangements and I thank them very, very much."



"I was **unable to travel** that day, so **a teleconference was arranged** and that all went very well."

"I was **given all of the information I needed**, as well as conversations with several EMA staff before the teleconference to check that I knew the minimum I had to read and respond to in the meeting. I was also told I could read more if I had an interest, and **at all times I was referred back to during the meeting and people were very good at stating which document they were talking about**, which page, etc. all very good."

Comments from patients and consumers

"Some speakers give their **presentations without having sent them before** so participant can't take notes into the printed out documents."



"There were **sometimes issues with EUDRA download links**, leading to delay access to documents and extra time for project assistants to ensure documents were sent."

"**Flight tickets came very late** (day before). The room number of the meeting was different then on the invitation, because I was almost too late."

"Invitations, meeting services generally ok, **expenses terrible**. It is trying to get the money back out of you is the problem."

Comments from healthcare professionals



"Efficient, very quick and helpful. An example for many other organisations."

"More than perfect, **all is very well thought over.**"



"But, again, **lots of paperwork**. Sometimes - when looking at the agenda - I wonder about the relevance of some points on the agenda."

"We have **received invitations very late** and we don't have the time for reactions."

Future participation

In Figure 29, we see the likelihood of future participation in EMA expressed by patients, consumers and healthcare professionals. The majority of respondents expressed a high likelihood that they would participate in EMA activities in the future, demonstrating the high importance placed on these activities as well as showing commitment to contributing human medicines regulatory activities with real life experience or clinical practice.

Figure 29: Likelihood of future participation

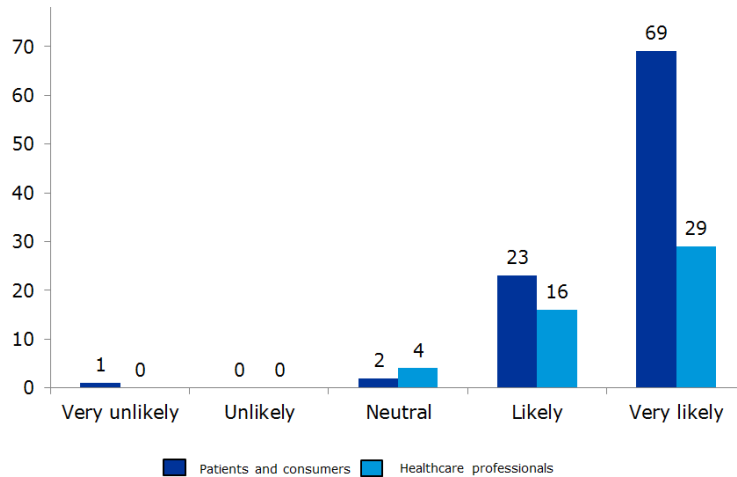


Figure 30: Comments from patients, consumers and healthcare professionals

*"Excellent responses by people working there. The technology could be improved. EMA's role is important so I **would encourage more patients to join**. However it's very technical and hard to understand for patients."*

*"I would appreciate to be allowed to **discuss the application papers with other patients or expert**."*

*"It depends on the **time commitment as well as the subject matter**. With no backfill the day job awaits!"*

Other comments

Other comments from patients and consumers

"Better define patient roles and responsibilities (e.g. better understanding of confidentiality) and perhaps provide some short training or mentoring at start."

*"The timeliness were very tight and the process was quite rushed. It **would be beneficial** to have appropriate time available to complete the process."*

"I would encourage patients to be involved in every session. It is invaluable to have someone there who can present the view from the patient perspective."

"I would suggest that the raw data should be sent out 4 days in advance. Given some of the arguments were still being formulated, those could come 48 hours prior to the meeting."

Other comments from healthcare professionals

"Overall, I am convinced that both sides EMA and HCPs take advantage. A further improvement could be pre-meeting dissemination of specific questions related to the topics on the agenda in order to stimulate discussions and to get a closer figure of opinions of the various stakeholders."

"It seems to me that the EMA is now more seeking input and advice from the HCP than before, with the aim to better fulfil patient needs."

"When looking for input it may be helpful to set out the time commitment and the minimum level of expertise required. Some may be more willing to engage on particular topics if the commitment was explicit and the confidence to engage supported."

Conclusions and way forward

The satisfaction survey of patients, consumers and healthcare professional overall shows high levels of satisfaction. Based on analysis of comments, the following points were considered as a way forward to continuously improve the initiative of patient involvement in EMA.

1. Maintain high standards of involvement and integration of patients, consumers and healthcare professionals across wide range of EMA activities and continuously aim for improving those standards.
2. Explore ways to streamline Declaration of Interest (DoI)/registration process.
3. Look at providing additional *one-to-one* support prior to the activity, especially regarding the involvement in scientific committees.
4. Expand current educational and training materials – EMA Basics, webinars etc.
5. Endeavour to send out the documents earlier to allow adequate preparation.
6. Explore how to improve feedback for each activity.
7. Explore further options on financial support.