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

Annual report 2015



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## Message from Noel Wathion – Deputy Executive Director



“Over the past 20 years, I have seen many changes at the Agency, in particular in relation to the Agency’s interactions with civil society representatives, which have evolved and strengthened over time. This has allowed the Agency to move from ‘interacting with’ to ‘engaging with’ these stakeholders.

It has been a pleasure to see over these years how a better integration of these stakeholders into the Agency’s work, and in particular the scientific review process for the authorisation and maintenance of medicines, is enriching the scientific debate and making the scientific output more robust.”

## **Executive summary**

2015 was an important year in pharmaceuticals as it was both the 20<sup>th</sup> anniversary of the establishment of the European Medicines Agency and the 50<sup>th</sup> anniversary of the pharmaceutical legislation. The EMA marked this anniversary with a scientific conference entitled '*Science, Medicines, Health: Patients at the heart of future innovation*' (see Section 1.3.). Since its creation, the EMA has continued to evolve and adapt to legislative changes resulting in expansion of its area of work to encompass areas such as rare diseases, herbal medicines, medicines for children and advanced therapies.

Concurrent with this, the Agency also expanded activities where patients and healthcare professionals could be involved (Figure 1). Patients are members of most scientific committees and a pilot was launched in 2014 to also include them in oral explanations at the Committee for Human Medicinal Products (CHMP). The timeframe planned for the pilot has been extended to allow for more examples. In addition to this, discussions with the Herbal Medicinal Products Committee (HMPC) have begun regarding the involvement of patients as observers during a plenary meeting, establishment of a group of experts to review herbal summaries as well as taking part in ad hoc consultations by the committee.

The question is no longer how to involve these key stakeholders but how best to support their involvement, increase the recognition of the work they do and in some instances measure the impact of their contributions on regulatory outcomes.

To address some of these questions and more, specific topic groups were created in the context of the Patients' and Consumers' Working Party (PCWP) and the Healthcare Professional's Working Party (HCPWP) (see Sections 2.2.2.2. and 3.2.2.2.).

These groups have been established to address the following topics:

- Measure the **impact/value** of patient involvement in EMA activities
- Acknowledge and **promote visibility** of patient input into the Agency's activities
- **Training** for patients involved in EMA activities
- Involvement of **young people** in EMA activities
- **Social media**
- **Risk minimisation measures** and assessment of their effectiveness
- EMA/CHMP/PRAC projects on **information on medicines**
- **Academia**, learned societies and healthcare professional organisations

The creation of the topic groups has been an important new way of expanding EMA collaboration with the members of the working parties outside the plenary meetings and also as a way of interacting with more of the eligible organisations on matters of mutual interest. The topic groups will draft recommendations to be brought back to the working parties and the EMA on the various topics that are considered to merit extra attention.

It has been identified as important to broaden the scope of the current stakeholder groups in order to be certain to understand and best capture the needs of those affected by regulatory decisions, as well as those not yet involved but who play an important role in medicines development and public health.

For these reasons, discussions have begun and are ongoing regarding the involvement of young people and finding methods for including their voice in regulatory processes. A specific PCWP topic group has been created and is composed of members with experience in this area; they are currently exploring the best way forward. Similarly in the context of the 20<sup>th</sup> anniversary, a dedicated session on involving young people in the evaluation of medicines for children was convened (1.3.1.).

This year also saw targeted contact being made with general practitioners and specialist nurses. The importance of the perspective of these groups on public health and the impact of regulatory decisions is clearly recognised and the Agency is liaising to establish practices to best involve them in its work.

The importance of academia as a source of innovation and enrichment of the product pipelines of larger companies has been established<sup>1</sup> and interactions with this group have also been brought to the forefront this year. Work has begun to determine how best to work with academia, what their needs are and how best to address them.

The Agency is committed to continuing to provide information and education to patients and healthcare professionals on topics of interest and relevance. Several information sessions and workshops were held over the course of this year. Of particular relevance were the information session on biosimilars and the workshop on risk minimisation measures. The annual training day of the EMA has been restructured to ensure more hands-on experience with the activities where patients are involved at the EMA (see section 2.4. ). Background information related to the various activities of the EMA is provided in the patient and healthcare professional specific web pages thus enabling more time for practical experiences and opportunities to ask questions to EMA staff involved in particular procedures.

### ***Future steps***

As already described the challenge is not in establishing the involvement of stakeholders in the medicines development process but maintaining and improving these processes. Expansion to different stakeholder groups including young people and general practitioners will be the focus of the coming years.

The Framework for Interaction between the European Medicines Agency and Healthcare professionals is now approaching five years, since its adoption in 2011. We have identified a need to further clarify the different aspects of their activities, in particular clinical practice and the development of therapeutic guidelines, and the role of learned societies in academic research and education. In this context, the healthcare professionals' framework needs to be revised and a specific framework of collaboration with academia will be developed (see Section 3.2.2.).

Several actions described in the revised framework of interactions with patients and consumers have been implemented and are being developed further. Establishing a pool of experts remains an important aspect to our work in order to ensure participation throughout the lifecycle of medicines. Activities will be maintained to increase awareness of the work of the Agency and to continue to develop capacities by improving the training and support offered to those interested in learning more as well as those invited to participate in EMA activities.

The EMA also needs to 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.

The EMA is moving closer to the implementation of public hearings and 2016 will see them put into practice.

Finally, in all its activities it remains critical to identify the right individuals to participate in procedures and web-based tools to support this process will be created.

This report was circulated to the joint PCWP/HCPWP and was presented to the Management Board during its meeting on 16 June, 2016.

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<sup>1</sup> <http://www.nature.com/nrd/journal/v13/n2/full/nrd4232.html>

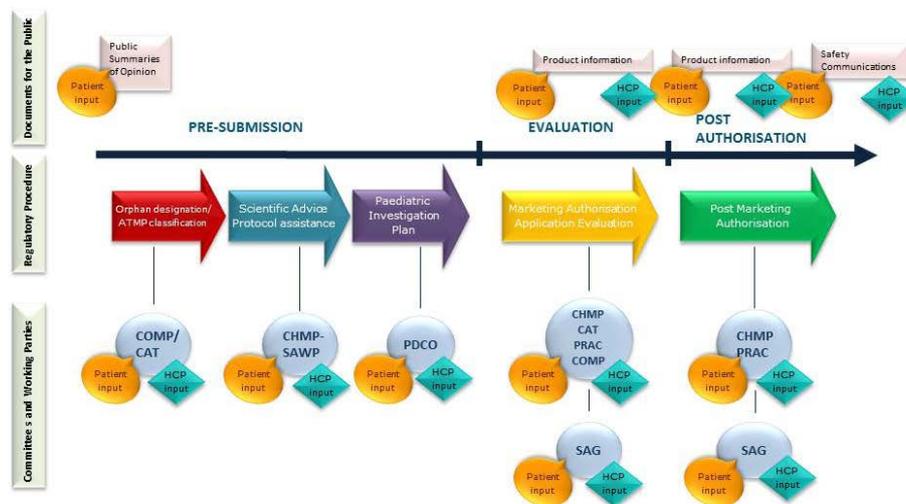
# 1. Patients, consumers and healthcare professionals: common areas of interest and collaboration

## 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).

**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 common areas of 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. Eligibility requirements for organisations working with EMA

The 'eligibility criteria for organisations' were revised, and adopted by the EMA Management Board as described in the 2014 annual report. A [guidance document](#) has been prepared that outlines the revisions and provides additional detailed information to organisations regarding these changes.

The Patients' and Consumers' Working Party and the Healthcare Professionals' Working Party were fully involved throughout the process in 2014 as well as during the joint working party meetings in 2015 (see section 1.5. ) and at the meeting with all eligible patient organisations. The main changes to the criteria relate to a better spread of funds received, the proportion of funding that organisations can receive from a single pharmaceutical company, full transparency in the publication of their yearly funding sources and proportion of funds received as well as adherence to a 'code of conduct/rules with regards to the relations of an organisation with industry'. The revised criteria come into effect as of end of 2015.

It is important to remember that in addition to the eligible organisations, the EMA also works with a diverse group of organisations outside the eligible organisations, when needed, on a case by case basis with respect to disease-specific questions (listed in Table 9 and Table 18).

### **1.3. EMA 20<sup>th</sup> anniversary conference**

The European Medicines Agency celebrated its 20<sup>th</sup> anniversary with a [conference](#) entitled '*Science, Medicines, Health: Patients at the heart of future innovation*'. The conference brought together members of the European Commission, the US Food and Drug Administration (FDA), patients, healthcare professionals (HCPs), payers, investors, academics as well as representatives from other regulatory agencies. While a review of the past two decades, the successes, growth and achievements of the Agency, it was also an occasion to look at the challenges of the future.

Guido Rasi, Executive Director opened the meeting and highlighted three key words to describe the future – '*complexity, opportunity and patients*'. To elaborate further, he said that it is clear that the future of medicine will be more complex with increasing complexity of molecules, cells and tissues as well as technology for delivery of medicines. Diagnostic technology used to identify patients who will benefit from medicines and disease classification will also become more complex. However this complexity should not be perceived as a burden, but as a challenge that opens up opportunities.

As we head towards personalised medicines, opportunities arise for patient-driven approaches as opposed to market-driven approaches. There are new opportunities for academia, small to medium-sized enterprises, spin-offs and big pharma to be involved at the different stages of development.

EMA is very proud of what it has done to generate evidence in collaboration with patients. Guido Rasi said that "*it is one of the success stories of EMA as only they can really bring us the real benefit-risk assessment; they are entitled to teach us the added quality of life of any therapeutic approach*". He also said that EMA is committed to having patients fully integrated into the decision-making process.

Other presentations included those from Ladislav Miko (Acting Director General for DG Santé) and Margaret Hamburg (US FDA Commissioner), as well as a panel discussion that included perspectives of patients, payers, investors, academics and regulators.

The patient viewpoint was presented by Yann Le Cam (EURORDIS) who stated that innovation and the system at large are not meaningful unless a medicine reaches the patients. He encouraged a 'real dialogue' all along the process of medicines development with patients, healthcare professionals and payers in the same room including at the time of marketing authorisation. Each perspective will be different however an exchange of information about disease, the product, the evidence base and uncertainties is needed to ensure a continuum of good decisions in a currently fragmented situation.

The meeting was closed by Sir Kent Wood, chair of the EMA management board and the entire conference was recorded and available on the [EMA website](#).

#### **1.3.1. Internal awareness raising events for 20<sup>th</sup> anniversary**

In the context of the 20th anniversary of the Agency and in order to showcase the expanding remit of the EMA during that time, a series of talks for staff and scientific committee members were organised. Two of these are described below.

##### ***Involving young people in the evaluation of medicines for children***

A panel moderated by Dirk Mentzer (PDCO chair) was invited to debate the topic of involving young people in evaluation of their medicines. Panellists included June Raine (PRAC chair), Bruno Sepodes (COMP chair) as well as Pamela Dicks (Scottish Clinical Research Network), Rafal Zwierzewski (member of PCWP, representative of ECPC and cancer youth group leader in Poland) and Irina Rotariu (member of the Scottish Children's Research Network Young Persons Advocacy Group (YPAG)).

Each panellist was invited to present their viewpoints prior to opening the debate to the audience. June Raine began by stating that the question is 'not *if* but *how* we can better involve children and young people in making our medicines appropriate for them and their lives'.

Young people are best placed to comment on whether trials are acceptable in terms of timings, palatability of medicines and other aspects that could impact on recruitment.

Rafal Zwierzewski explained that in order to best involve them in EMA activities, it was first necessary to clarify the Agency's expectations of them as well as to propose concrete areas for involvement such as in regulatory and educational guidelines as well as in dissemination of information as young people are skilled in communication technology.

The final word of the panel prior to the open discussion went to Irina Rotariu who described two instances (Crohn's disease and flu treatment) where consultation with the young person's group altered the protocol proposed for the clinical trials. She stated that young people's input covers aspects that are more practical and less technical than some of the other experts but equally valid and with far reaching impact.

When asked if it would be too intimidating to address the whole committee, she replied no, but suggested to begin with a small group of young people meeting with a small group from the committee as a large group could be daunting. Although the audience appreciated that she was quite comfortable both during the lunch talk as well as earlier when addressing the Paediatric Committee.

*"Young people are best placed to comment on whether trials are acceptable in terms of timing, palatability of medicines and other aspects that could impact on recruitment."*  
Pamela Dicks  
(Scottish Clinical Research Network)

The take home messages were that communication is key and feedback following consultation is very much appreciated.

### ***The view from the sharp end: what patients and healthcare professionals can do for us***

In the last of the lunchtime talks, a panel comprising David Haerry (co-chair of the PCWP), Gonzalo Calvo (co-chair of HCPWP), Bruno Sepodes (COMP chair) and Isabelle Moulon (EMA Head of Patients and Healthcare Professionals Department) was moderated by Juan Garcia Burgos (EMA). The aim of this session was to give an overview of the experience that the Agency and its committees have in involving patients and healthcare professionals in its work. It also looked at the challenges ahead and future opportunities.

Juan Garcia opened the discussion by stating that in his opinion this was one of the most relevant aspects of the work of the EMA - incorporating the views of civil society in medicines regulation. Now at 20 years of age, it was a good opportunity to look back to see how experience and interactions have evolved over time.

Bruno Sepodes stated that contrary to the beliefs of some, not all patients want to have all products licenced and in some cases they have shown themselves to be tougher than regulators. David Haerry reminded us that this all started when people living with HIV requested expanded access and compassionate use at the FDA in the early 1980's. Interactions with EMA began early in its existence and regulators began to understand that it was useful to talk to patients. This led to the creation of the working party and involvement in workshops to capture patient input and real life experiences. Patients are now involved everywhere – all along the lifecycle.

Gonzalo Calvo said that although it should be considered to be 'normal' that healthcare professionals, patients and members of civil society should be part of the regulatory process, in 1995 this was not widely accepted. While healthcare professionals have always been part of regulatory process as

experts nominated by national agencies, it was not until later that their involvement on a permanent basis in scientific advisory groups (SAG) where they advise committees became possible. Many questions were raised on the capacity under which they would be invited as well as issues of conflicts of interest however their added value quickly became evident.

The EMA has implemented a framework for interaction with healthcare professionals, as it had previously with patients, which then led to the creation of the working party. He went on to say that many things were being done in HCPWP from channelling information on EMA regulatory decisions to medical and pharmacy groups and seeing how this information impacts in the real clinical setting to exploring how information can be improved. He concluded with a personal reflection as a former CHMP member saying that he 'did not realise that decisions were impacting on society (on patients and on clinical setting)'. It is clear when considering these decisions that the people most affected should be involved.

The final word was given to Isabelle Moulon who reiterated that interactions between patients and regulators were triggered by people living with HIV. In first meeting with HIV patients, regulators were taken by surprise to hear that the patients were able to discuss their needs and to bring complex scientific discussions to the debate as well as to exercise common sense.

Patients have the power to change things and their work has been widely recognised, we have to move forward and no discussions can take place about medicines and treatments without patients.

In the case of healthcare professionals the situation is more complex, while their presence in scientific committees is unquestioned; it is frequently the dimension of clinical practice that can be forgotten. This concerns not only physicians and specialists but pharmacists and nurses too.

She concluded by saying that clinical experience of healthcare professionals as well as the experience of patients in living with a disease are as important as the scientific aspect of the development of medicines.

#### **1.4. EU Medicines Agencies Network Strategy to 2020**

For the first time, the EMA and the Heads of Medicines Agencies (HMA)<sup>2</sup> have developed and adopted a [common strategy](#) for the European medicines regulatory network to reflect the need for a coordinated approach to address the multiple challenges and opportunities facing the network. The strategy outlines joint key priorities and a high-level roadmap to achieve these.

The strategy focuses on specific areas where collaboration can make a real difference to human and animal health in the EU to 2020 and builds on the [EMA roadmap to 2015](#) and the HMA strategy document. Four strategic priority areas were identified - i) human health, ii) animal health and human health in relation to veterinary medicines – minimising the risk from the use of antimicrobials in veterinary medicine, iii) optimising operation of the network and iv) strengthening the global regulatory environment.

Separate multi-annual work programmes/implementation plans will be developed for EMA, HMA and the coordination groups for mutual recognition and decentralised procedures (CMDh and CMDv), which will provide detailed information on the work of each component of the network and describe how the strategy will be taken forward.

The common strategy makes a special mention of dementia as well as special populations that include children, elderly and rare diseases. Ongoing issues regarding timely access and appropriately

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<sup>2</sup> The Heads of Medicines Agencies is a network of the Heads of the National Competent Authorities whose organisations are responsible for the regulation of medicinal products for human and veterinary use in the European Economic Area.

developed medicines with information supporting their use are mentioned as well as ensuring that existing flexibilities for access are used to their maximum potential via adaptive pathways. Strengthening collaboration with HTA/pricing and reimbursement bodies, healthcare professional and patient representative bodies is a key factor to ensure success.

Patient values and preferences into the scientific review process are specifically mentioned and the impact on considerations of benefit risk across the network (please also see 2.6.2.). Optimal implementation of the Clinical Trial Regulation and greater use of real world databases and increased transparency regarding data that underpin regulatory decisions were also highlighted as key issues in this joint strategy (Section 1.8.).

Noel Wathion (EMA) first presented the draft strategy to the working parties in the March joint meeting and returned in June to inform the PCWP and HCPWP of the public consultation, inviting them to provide comments.

All comments received were considered by EMA and the HMA and the final [EU Network Strategy to 2020](#) was published in December 2015.

### **1.5. 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 with both working parties at the same time.

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 2015, three PCWP and HCPWP joint meetings were organised ([4 March](#), [4 June](#), [17 September](#)). Summaries of a selection of topics discussed during these are provided below.

#### **1.5.1. Working party topic groups**

In response to discussions during [PCWP and HCPWP meetings](#) as well as with the working parties co-chairs, a proposal was put forward to create specific groups on topics of mutual interest to EMA and the working parties. The topics would be specific to one or other working party or combined where appropriate. This would provide more time for ideas to be brainstormed in smaller groups between the plenary meetings, promote further discussion of specific topics as well as to enable better utilisation of time during the face-to-face meetings and would further stimulate participation and engagement.

The initial topics selected are listed below along with the specific working party they are assigned to:

- Measure the impact/value of patient involvement in EMA activities (PCWP)
- Acknowledge and promote visibility of patient input into the Agency's activities (PCWP)
- Training for patients involved in EMA activities (PCWP)
- Involvement of young people in EMA activities (PCWP)
- Social media (PCWP/HCPWP)
- Risk minimisation measures and assessment of their effectiveness (HCPWP)
- EMA/CHMP/PRAC projects on information on medicines (HCPWP)

- Academia, learned societies and healthcare professional organisations (HCPWP)

The proposal was welcomed by PCWP and HCPWP members and topic groups were launched in June 2015; more information about each topic group is provided in the working party specific sections (2.2.2.2. and 3.2.2.3. ).

### **1.5.2. Member's voice**

As part of the ongoing initiative to encourage contributions from working party members in the context of learning from each other, a section entitled 'Member's Voice' was introduced during the joint PCWP and HCPWP meeting of September 2015.

The aim of the Member's voice is for members of patients', consumers' and healthcare professionals' organisations to share practices and projects they are working on, of interest to the EMA and the other organisations. This initiative was welcomed by the members of the working party and will be continued as a routine part of the agenda.

The first to share their activities was the patient organisation, the European Multiple Sclerosis Platform (EMSP) who presented the European Register for Multiple Sclerosis (EUREMS). The objectives include effective cross-border cooperation of national registries, data pooling and centralised data analysis in the MS field.

Representing healthcare professionals, the European Society for Medical Oncology (ESMO) presented results from the ESMO Antineoplastic Medicines Survey, showing that there are disparities across Europe with respect to access to cancer medicines. Medicines shortages affect several essential and inexpensive medicines that have been on the market for a while. Inequalities exist in availability and costs across Europe in particular for newer more expensive medicines.

### **1.5.3. Strategy to measure the impact of pharmacovigilance decisions**

The Pharmacovigilance and Risk Assessment Committees' (PRAC) draft strategy aimed at measuring the impact of pharmacovigilance processes and decisions was [presented](#) during the September meeting.

The actions available to regulators when new safety information arises include updating product information (Summary of Product Characteristics (SmPC), package leaflet and labelling), informing patients and/or healthcare professionals via letters or educational material, review of the benefit-risk profile of the medicine via a referral procedure or restricting access to the medicine.

The main goals of the draft strategy are to determine whether these regulatory actions have been successful in minimising risk and to identify barriers and enablers for ensuring effectiveness of the processes and actions, in order to strengthen and improve pharmacovigilance.

Patients and healthcare professionals play key roles in pharmacovigilance as they are those impacted by regulatory outcomes. They are the actors who ultimately achieve safe and rational use of medicines. In this light, the working party members were amongst the first to receive information regarding the planned strategy to measure the impact of pharmacovigilance. A 'virtual collaboration group' was set up with interested PCWP and HCPWP members who will provide input into methods to survey engagement in pharmacovigilance and trust in the system

#### **1.5.4. New pharmacovigilance systems and services**

In 2014 the PCWP and HCPWP representatives were updated on changes on the EU Pharmacovigilance legislation that came into effect in July 2012. An update was provided during the September meeting on achievements, the next steps of Article 57 and [EudraVigilance](#) and how this will benefit stakeholders. Information systems to enhance pharmacovigilance are foreseen, in particular to support the collection, management and analysis of data, information and knowledge.

Activities put into practice in 2015 included medical literature monitoring (MLM) as part of EudraVigilance, a PSUR<sup>3</sup> repository and the introduction of pharmacovigilance fees. The benefits for stakeholders and future steps were explained. Major deliverables from all these projects are scheduled throughout 2016 and 2017 to support business activities of the revised pharmacovigilance legislation and to improve the relevant business functions to maximise the benefits for stakeholders. An update will be provided to the working parties over the course of 2016.

#### **1.5.5. Enhanced early dialogue to foster development and facilitate accelerated assessment (PRIME)**

A new [scheme for priority medicines \(PRIME\)](#) was presented to participants during the September meeting. The aim of PRIME is to provide enhanced scientific and regulatory support to companies developing medicines that may offer new therapeutic options to patients who currently have no treatment options, or a major therapeutic advantage over existing treatments. During the presentation, the participants raised points for clarification and were reassured that these would mostly be covered by the [reflection paper](#), which was open for public consultation from late October until December 23<sup>rd</sup>.

The objective is to optimise development and facilitate the accelerated assessment of new priority medicines to benefit patients as early as possible and to encourage developers to focus on medicines with a potential significant benefit. Early dialogue, enhanced interactions, building on existing regulatory processes such as scientific advice will all be reinforced in order to optimise the generation of robust data and the accelerated assessment procedure to improve timely access for patients to priority medicines.

PRIME was launched in the first quarter of 2016 and updates will be provided on developments, including any possibilities for involvement.

### **1.6. Workshops and Information Sessions**

#### **1.6.1. Biosimilars information session**

A biosimilar is a biological medicinal product containing a version of the active substance of an already authorised original biological medicinal product (i.e. the reference medicinal product). A biosimilar demonstrates similarity to the reference medicinal product in terms of quality characteristics, biological activity, safety and efficacy based on a comprehensive comparability exercise.

These products are thoroughly evaluated by regulators and have the same surveillance as any other product once authorised. There are many misconceptions regarding biosimilar medicines and more education and better communication is needed to inform patients and healthcare professionals.

A need to improve the understanding of healthcare professionals and patients of biological medicines, including biosimilar medicines, was identified through the European Commission's [information paper](#)

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<sup>3</sup> [Periodic safety update reports \(PSUR\)](#): questions and answers

(2013). As biosimilars present a number of challenges, it is important that patients and healthcare professionals are fully informed on how these medicines are defined, assessed for marketing authorisation and monitored once on the market. Increasing understanding would serve as a means to increase confidence in these medicines.

This [information session](#) brought together members of the Patients and Consumers Working Party as well as the Healthcare Professionals' Working Party and regulator representatives. In preparation of the session the working party members were consulted to enable the presentation of their concerns regarding biosimilars and to allow the workshop to address particular questions and issues from patients and healthcare professionals.

The session was aimed to provide information on how these medicines are evaluated as well as on the current and foreseeable challenges and opportunities. The meeting was opened by Sir Kent Woods, chair of the EMA Management Board. He said that while currently there are 19 biosimilars (similar biological medicinal products) authorised in the EU, biosimilars are gaining importance because an increasing proportion of innovative products are biologics and expiry date of data exclusivity is approaching. He said that we could expect the numbers of biosimilars to increase.

The agenda also included presentations on key scientific and regulatory concepts related to biosimilars such as comparability, immunogenicity and post-authorisation studies and concluded with a look at what is needed to better understand these medicines. In addition, the viewpoints and concerns of patients and healthcare professionals were presented, based on an initial consultation of the working party members as mentioned above.

The concerns from patients' and consumers' organisations, presented by David Haerry (PCWP), were related to aspects such as managing the perception of biosimilars and creating understanding between generics, biologicals and biosimilar products and switching from products. They also saw a cost saving potential for biosimilars which should be further discussed with HTAs. The concerns of healthcare professionals' organisations, presented by Michel Delvaux (HCPWP), primarily related to issues such as how similar biosimilars are used and their interchangeability, the licensing process, safety, education and information and economic aspects.

In an evaluation following the session, 35 of 36 (97%) responded that the session contributed to their understanding of the science behind biosimilars and the regulator's role. Understanding of the challenges faced by patients and healthcare professionals in relation to biosimilars improved for 32 out of 36 (89%), while 86% felt that the session provided useful information for future activities within their organisation on the topic of biosimilars.

*"I feel I am now able to inform my fellow volunteers in the organisation I work for about the basic principles of biosimilars."*  
Patient participant

### **1.6.2. Developing a proactive approach to the prevention of medicines shortages due to manufacturing and quality problems**

Shortages of medicines are a global problem and are also increasingly impacting patient care in the European Union (EU). The causes of medicine shortages include economic, business, political, manufacturing and distribution issues. However, the EMA is mainly involved with shortages due to manufacturing or Good-manufacturing-practice (GMP) compliance problems.

Further to the EMA's workshop on shortages organised in 2013, the Agency convened a second stakeholder meeting in October bringing together national competent authorities, industry and patient

and healthcare professional representatives to discuss recent initiatives and to reflect on possible further actions to proactively manage shortages.

The patient perspective (presented by Francois Houyez) was that despite the work done to date, shortages continue to impact on patient care and patients are not well informed about shortages, their duration and resulting substitutions. There is an urgent need to better inform patients about shortages and how it impacts on their care but also to better simulate and pre-emptively manage a potential shortage.

The extent of the problem of shortages as experienced by healthcare professionals, was illustrated by the results of two surveys: the 2014 report of the European Association of Hospital Pharmacists (EAHP) on medicines shortages in European hospitals (presented by Richard Price) and a 2015 survey of community pharmacists undertaken by Pharmaceutical Group of the European Union (PGEU) (presented by Jamie Wilkinson). Pharmacists and other healthcare professionals are usually not provided with information about why a shortage has occurred, and when the situation might improve, which means that they are unable to give assurance of future supply, creating uncertainty and anxiety for patients. More effort needs to be put into quantifying shortages as well as communicating shortages to healthcare professionals.

The full [report](#) of the meeting outlines the contributions of all the stakeholders attending as well as the way forward for the regulators.

### **1.6.3. Workshop on the development of new medicinal products for the treatment of ulcerative colitis and Crohn's disease**

Ulcerative colitis and Crohn's disease are the two most common types of inflammatory bowel disease. They are chronic auto-immune diseases that cause considerable ill health and mortality in patients, and affect more than two million people across the EU. Moreover, both ulcerative colitis and Crohn's disease patients have an increased risk of developing colon cancer.

A [workshop](#) was held in light of the ongoing revision of the guidelines on the development of medicinal products for the treatment of Crohn's disease and for the treatment of ulcerative colitis, respectively. Patients and healthcare professionals attended the meeting.

The objectives of the workshop were to discuss the available data on validity and feasibility of mucosal healing as a primary measure of efficacy in adults and children, provide a forum for discussion on study designs and possible claims for new substances and to receive input on the criteria for extrapolation possible from adults to the paediatric population.

### **1.6.4. Demonstrating significant benefit of orphan medicines: concepts, methodology, and impact on access**

Significant benefit is one of the criteria for orphan designation and is defined as '*a clinically relevant advantage or a major contribution to patient care*' and is unique to the European orphan legislation.

This [workshop](#) held in December aimed to discuss the concept of significant benefit by examining existing methodologies for comparative efficacy and effectiveness and for major contribution to patient care, including patient preferences. This includes how these could be applied in demonstrating significant benefit at marketing authorisation as well as the impact of significant benefit on health-technology-assessment (HTA) evaluation, pricing decisions and patient access.

The workshop brought together European regulators, HTA bodies, the pharmaceutical industry, payers, patients, health care professionals and academics. Perspectives from patients and healthcare

professionals were presented by EURORDIS and ESMO, respectively. In addition, representatives from the Committee for Orphan Medicinal Products (COMP), Committee on Medicinal Products for Human Use (CHMP), Paediatric Committee (PDCO) and the Scientific Advice Working Party (SAWP) were also present.

### **1.6.5. Workshop on risk minimisation measures**

A risk minimisation measure is a strategy to prevent or reduce the occurrence or severity of an adverse drug reaction when a medicine is used in daily practise. The goal of the measures, as recognised during the workshop, is to reduce harm from use of a medicine as evidence is gathered in clinical use, reducing the uncertainties that exist at time of market authorisation. A variety of tools for risk minimisation measures are currently available and are continuously developed building upon technological advances.

A [workshop on risk minimisation measures](#) was organised as part of the joint PCWP and HCPWP meeting in September 2015. The aim was to review existing risk minimisation measures and how they are supported by the regulatory environment, what is working well with the development, implementation and evaluation of risk minimisation measures in real life clinical practice as well as identifying what can be improved. The workshop brought patients', consumers' and healthcare professionals' representatives, academics, members of EMA's scientific committees and EMA staff together.

The importance of the contribution from healthcare professionals and patients during the development of risk minimisation measures is to ensure that risk minimisation measures are adequate, balanced, feasible and do not create an undue burden to the healthcare system. In addition, not only do the measures themselves evolve, but risk minimisation measures are also an evolving area of medicinal sciences with a need for universally agreed standards and approaches.

Healthcare professionals and patients pointed out that health literacy of patients may differ between patients and should be taken into account when writing documents and communication.

The PCWP co-chair David Haerry reminded us that we must work as a team, which includes pharmacists, healthcare professionals, patients, regulators, industry and researchers. His comments were followed by those of the co-chair of the HCPWP, Gonzalo Calvo, who said that while there was a clear need for healthcare professionals to understand the science behind risk minimisation, conversely regulators also need to understand the limitations of the measures they impose in clinical practice. His suggestion was to improve proactive interaction between working party and regulators to overcome these limitations.

The HCPWP Topic Group on risk minimisation measures will take forward the workshop's outcome in terms of engagement with healthcare professionals when designing risk minimisation measures and measuring their impact; other proposals from the workshop will be addressed through different work plans and research activities.

A [report](#) on the workshop has been written and published on the EMA website.

## **1.7. Increasing understanding and awareness of EMA activities**

### **1.7.1. 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.

### 1.7.2. 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.

### 1.7.3. 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 2015, 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 CHMP opinion /CMDh position
- Information on shortage of medicines (please refer to 3.2.1. for more details)

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 DHPC<sup>4</sup>);

***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.

***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 the above 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. Throughout 2015, 12 issues were published and are available in the News and Events tab on the homepage under the Newsletter heading.

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<sup>4</sup> DHPC: Direct Healthcare Professional Communication

#### **1.7.4. External queries**

Every year the Agency receives external queries from individuals through the online information request form on the EMA website. In 2015, the Agency responded to 482 queries from patients/consumers and 231 from healthcare professionals. Questions were mainly related to centrally authorised products, non-centrally authorised products and referrals. Approximately 19% of the queries were received from non-EU countries.

#### **1.7.5. Review of risk management plan summaries- *outcome of pilot phase***

In March 2014, the Agency launched a one-year pilot regarding the publication of risk management plan (RMP) summaries and the outcome of the pilot was presented to the PCWP.

The aim of publishing these summaries was to provide a new information resource and increase public access to relevant information on medicines. The summaries would complement other information on medicines such as the summary of product characteristics (SmPC), package leaflet, EPAR summary and product assessment report.

Eligible organisations representing patients, consumers and healthcare professionals were surveyed on the desirability, utility and clarity of these documents. Each summary includes an overview of the disease and its epidemiology, a summary of the benefits and main safety concerns, important identified and potential risks as well as a summary of risk minimisation measures for each of the safety concerns and the planned post-authorisation development plan.

During the pilot, 84 RMP summaries were published and analysis has shown that there is interest in these documents and it can be concluded that they are valuable to those requiring additional background to the package leaflet's safety information.

The publication of RMP summaries in the format used during the pilot phase will cease for new medicines that receive a CHMP opinion from January 2016 onwards. The new summaries will gradually start being produced once the new RMP template is finalised (after the public consultation). In the meantime, information on RMPs will continue to be made publicly available in CHMP assessment reports, which are published as part of the European public assessment reports (EPARs) for each medicine centrally authorised.

### **1.8. Contribution to EMA transparency initiatives**

#### **1.8.1. EMA policy on publication of clinical data**

The EMA policy on publication of clinical data (also known as Policy 070) entered into force on 1 January 2015 and applies to clinical reports contained in all marketing-authorisation applications submitted on or after this date.

To help stakeholders understand the requirements and prepare for the publication of clinical reports, EMA has organised a series of discussions and consultations on the work processes.

Two sets of guidance are of particular importance:

- identification and redaction of commercially confidential information in clinical reports submitted to the EMA for publication;
- anonymisation of clinical reports.

Three meetings took place in 2015 to ensure communication of important information to stakeholders. A [webinar](#) was held in June to explain the principles for the submission of redacted clinical reports, the redaction consultation process, as well as the presentation of guidance on what is not considered as commercially confidential information and on the anonymisation and redaction of personal data in clinical reports.

There was a follow-up in July, with a [meeting](#) with representatives from patients' and healthcare professionals' organisations, academia, pharmaceutical industry associations, National Competent Authorities and non-governmental organisations to gather their views on the draft guidance documents (mentioned above). A further meeting entitled 'the [Second stakeholder meeting on the implementation of the EMA's policy on publication of clinical data for human medicines](#)' was held in September. Patient organisations, healthcare professionals, academia, representatives of National Competent Authorities and pharmaceutical industry associations attended this meeting. The aim was to further inform and consult on the two sets of guidance under development in the context of the policy.

EMA also presented new technical guidance on the procedural aspects related to the submission of the clinical reports as well as the workflow of the redaction consultation process and the overall process leading to publication. For further information, please refer to the following webpage: [Publication of clinical data](#).

### **1.8.2. Clinical trial portal and database**

According to the Clinical Trial Regulation, the EMA is responsible for the development and maintenance of a clinical trials portal and database, which will serve as the source of public information on clinical trial applications assessed, and clinical trials conducted in the EU, from the time of decision to authorise a trial up to the finalisation of those trials and inclusion of their results in the database. The portal and database are key instruments to ensure transparency of clinical trials as they will allow a number of stakeholders to complete a wide range of processes using the same system.

A public consultation on how the transparency rules of the European Clinical Trial Regulation will be applied in the new clinical trial database was launched in January 2015 and concluded in February 2015. The aim of the public consultation was to seek the stakeholders' view on the application of exceptions in relation to the transparency provisions of the European Clinical Trial Regulation. In this way the regulation should strike the right balance between respecting patients' and doctors' needs and the public's entitlement to extensive and timely information about clinical trials and developers' and researchers' need to protect their investments.

The results of this public consultation were discussed during the joint March and June meeting of the PCWP and HCPWP. Based on the public consultation it was proposed to categorise the clinical trials in 3 groups: i) pharmaceutical development; ii) therapeutic exploratory and confirmatory; and iii) therapeutic use, in order to facilitate a system for publication of data and documents that is simple, predictable and automatic. In October 2015 the appendix, on disclosure rules, to the "[Functional specifications for the EU portal and EU database to be audited](#)" was published. The timeframe, endorsed by the EMA Management Board, foresees that the portal and database are planned to be available for an independent audit by August 2017. Members of the PCWP and the HCPWP attended two stakeholder meetings at EMA contributing to the design and development of the portal and database.

### **1.8.3. Clinical trial lay language summaries**

In addition to the information provided by the database, the legislation provides for a summary of the results and a lay language summary to be published 12 months after the end of the trial. During the

PCWP/HCPWP '[patient's voice](#) in the evaluation of medicines' workshop in 2013, a need for lay language summaries of trials to enable patients to understand the results was further emphasised as well as briefings where outcomes are sensitive or of concern.

Guidance and templates for the sponsors will be developed in order to establish common grounds of what should be included. The NHS, on behalf of the United Kingdom, will be leading in the preparation of this guidance and sought the input of the PCWP, as requested by the European Commission. They consider increasing transparency and making perceptions of health research more accurate as important goals of the summaries. The work on this guidance will build on existing work done.

During the joint meeting of June 2015 a presentation was made on lay language summaries by Amanda Hunn of the English National Health Service (NHS). The sponsor is responsible for submitting the summaries within 12 months however there is no capacity to review these summaries due to the high volume.

A task force of five patients was created to contribute to the development of the guideline and to work closely with interested stakeholders, including patients and consumers, industry representatives and academia. One suggestion made after the presentation was to involve healthcare professionals in the review and to include guidance for sponsors. In addition, members of the PCWP commented that other presentation modes could also be investigated, such as voice, webcast or multi-media.

## **1.9. Input on EMA pharmacovigilance-related initiatives**

### **1.9.1. Cross-committee task force on registries**

Patient registries may be requested to marketing authorisation holders as regulatory requirements for advanced therapies, medicines for paediatric use and orphan products. Independent, disease-based registries are a potential source of valuable safety and efficacy data on products as they enable data to be collected over a long period and allow comparison of products. The EMA is currently embarking on a [strategy](#) for patient registries.

In the context of the EU collaborative framework for patient registries described in detail in the 2014 Annual Report, six teleconferences and one face to face meeting at the EMA were held with the multi-stakeholder advisory group in 2015. Among the attendees were many patients and healthcare professionals and members of the PCWP and HCPWP.

### **1.9.2. Workshop on haemophilia registries**

The European Medicine Agency organised a two-day workshop on [haemophilia registries](#) in July 2015. This workshop provided a starting point to consider what regulators need from haemophilia registries.

Patients and healthcare professionals were invited to participate in the workshop. Input was provided on topics like what key data should be included, current practices, how to maximise the benefit for public health and desirability and achievability of synchronous registries. The aim of the workshop was to identify strengths and weaknesses of registries from the perspective of providing safety and efficacy data on products, and to consider approaches/initiatives to strengthen this.

### **1.9.3. Pharmacovigilance legislation: ninth stakeholders forum**

On 15 September the ninth stakeholders' forum took place to provide an update on key changes and aspects implemented since September 2015. Patients and healthcare professionals participated in the meeting. For further information, please refer to the following webpage: [Ninth forum](#).

The ninth forum was opened by Fergus Sweeney (EMA) who said that many of the tasks on the 'to do list' as described in the Pharmacovigilance legislation have now been achieved and that we are moving into era of using the new tools that have been established, which makes it a good time to reflect on the past but primarily to look forward to what the platform will allow us to do.

These tools include proactive monitoring, faster safety issue detection, faster warnings to users, more transparency and greater engagement with stakeholders, which allows us to develop and adjust how we do pharmacovigilance.

The PRAC member representing patients' organisations described the importance of involving patients in PRAC discussions. Representing healthcare professionals' organisations on the committee, the member said that the creation of the EMA has resulted in increased transparency and that more needs to be done to facilitate safety reporting by healthcare professionals and patients as prescribers and users of medicines.

An overview of the Pharmacovigilance and Risk Assessment Committee (PRAC) was presented, now having completed its first mandate. Different stakeholder perspectives (member states, industry, patients, healthcare professionals and academia) were all presented on the key achievements as well as areas for improvement and priorities for future work of the committee.

Some of the improvements highlighted included an update on Public hearings, to be launched in 2016, improving the process of referrals<sup>5</sup> as well as a discussion on maximising the utility of documents such as the Risk Management Plan (RMP) summaries and Periodic Safety Update Reports (PSUR) in terms of transparency supporting the work of the Agency.

### **1.10. 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.

#### **1.10.1. European Paediatric Research Network (Enpr-EMA)**

The European Network of Paediatric Research at the European Medicines Agency ([Enpr-EMA](#)) is a network of research networks, investigators and centres with recognised expertise in performing clinical studies in children. Patients and healthcare professionals are involved in the Coordinating Group.

The EnprEMA working group will liaise with the PCWP topic group on involving children in EMA activities (1. 2.2.2.2. ) as well as collaborating to develop a virtual European network of young people to input into the design and delivery of clinical research in children.

#### **1.10.2. ADVANCE workshop**

ADVANCE (Accelerated development of vaccine benefit-risk collaboration in Europe) is a project that aims to review, develop and test methods, data sources and procedures, which should feed into an efficient and sustainable pan-European framework that can deliver robust quantitative data for the assessment of the benefits and risks of vaccines that are on the market.

EMA is involved in this project by co-leading work package (WP) 1 in the development of a good practice guidance with a Code of Conduct for the planning, initiation, design, conduct and reporting of observational studies in the field of vaccines. The Code of Conduct will also support interactions

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<sup>5</sup> A referral is a procedure used to resolve issues such as concerns over the safety or the benefit-risk balance of a medicine or a class of medicines.

between different parties involved in studies on vaccines and provide confidence to health professionals and the public about the quality of their results. A public consultation of the [draft Code of Conduct](#) was launched by ADVANCE until 15th November 2015.

A workshop was also organised in the context of work package 1 (WP1) at the EMA in December 2015 where seven patients and healthcare professionals attended and contributed.

## 2. Interaction with patients and consumers

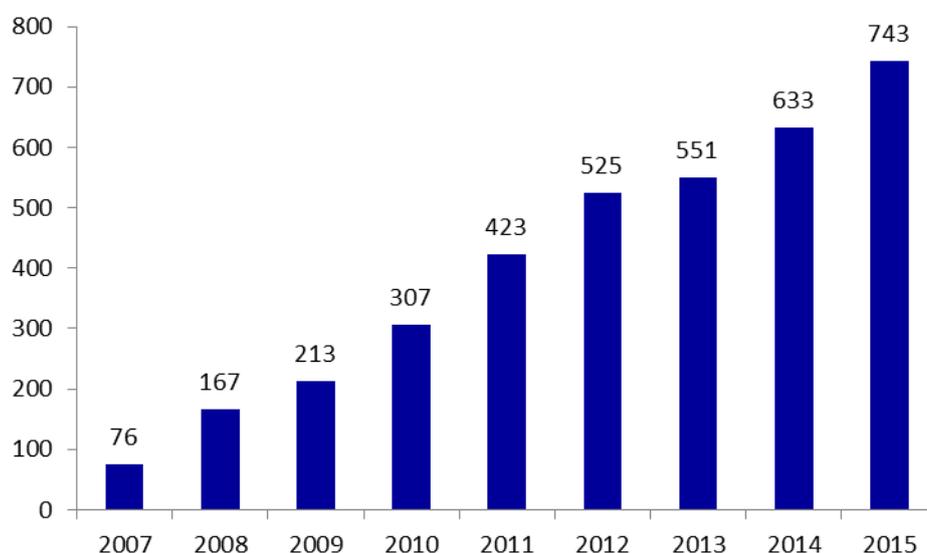
### 2.1. Introduction

Over the 20 years of the existence of the EMA, collaborations and interactions with patients has been well established. The activities where patients and consumers are involved have increased in diversity and in the numbers involved; this is described further below.

While constant review of best practices for these activities is always ongoing, the updated framework of interaction between the EMA and patients and consumers and their organisations clearly lay out the objectives. To achieve some of these objectives, topic groups were created as described in Section 1.5.1. The PCWP topic groups are described in this section and the meetings and conference calls organised resulted in the significant increase in patient numbers observed in 2015 (Figure 3).

The figure below illustrates the number of occasions where patients and consumers have been involved. The sharp increase from 633 in 2014 to 740 in 2015 is in part due to the creation of the topic groups.

**Figure 3: Overall number of patient and consumer involvement in EMA activities (2007-2015)**



#### 2.1.1. Implementation of objectives of the Framework for interaction

Within the Framework for interaction between the European Medicines Agency and patients and consumers and their organisations, revised in 2014, several objectives were clearly outlined. 2015 saw the establishment of several actions aimed at achieving these objectives with short, medium and long-term perspectives, depending upon the action.

The [Action plan](#) (in annex I of the Framework), highlights key actions that support the parallel five critical elements of the Framework (see Annual Report (2014)).

These key actions include:

- Maintenance of the network of EU patients' and consumers' organisations
- Establishment of a pool of individual experts

- Participation at key milestones during the lifecycle of medicines
- Building of capacities of patients and consumers invited to participate in EMA activities
- Increasing transparency on the involvement of patients and consumers and their organisations in Agency activities.

In the spirit of Participation, Consultation and Information, patients and consumers have been actively engaged to achieve these objectives. For a detailed description please see (section in PCWP regarding topic groups).

## 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 their 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**

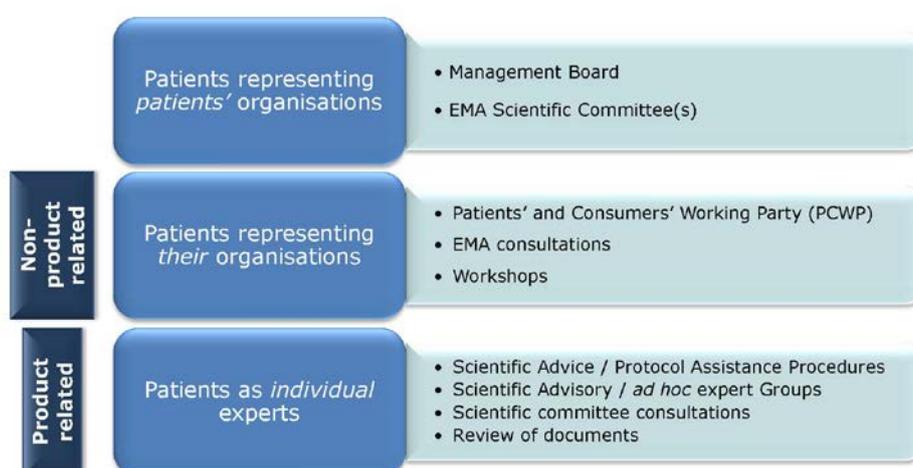
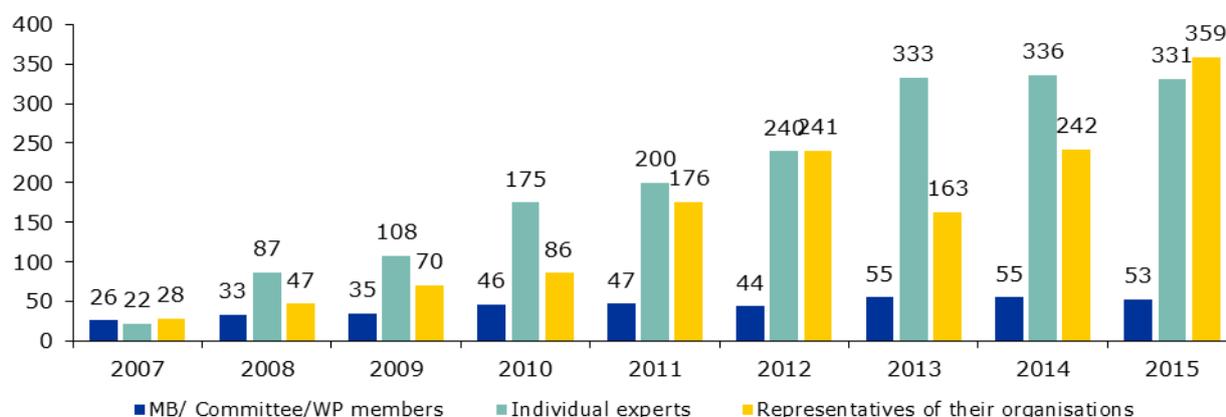


Figure 5 shows the distribution of the numbers of patients involved in the categories as mentioned above. More detail about each of these activities is provided in the corresponding sections below.

**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.

**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 1: Membership of patients 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

### ***Interactions with patients' organisations representatives from the EMA scientific committees***

In 2015, the Department of Patients and Healthcare Professionals initiated frequent and regular meetings with the representatives of patients' organisations from the scientific committees of the EMA. The purpose of the meetings is to provide support to these members where needed, be available for any clarifications as well as to assist in the identification of disease-specific patient experts for committee consultations.

The role of patients as members of the EMA scientific committees has been described in a [document](#) elaborated in 2009. Current plans exist to update and adapt this document to include the role of healthcare professionals and to highlight the added value of these members to the committees and to the work of the Agency.

## 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 16 alternates or observers (Table 2). The PCWP co-chair, David Haerry (EATG) is a patient representative and the EMA co-chair is Isabelle Moulon (EMA).

Two patient representatives are also members of the HealthCare Professionals working party to observe and introduce the patient perspective where necessary.

*'The creation of the topic groups was to ensure progress in specific areas outside the face-to-face meetings of the working parties. While each has different timelines for addressing their objectives and producing recommendations, some have already been very effective and have already implemented some of their objectives.'*

*(David Haerry, PCWP co-chair)*



**Table 2: Membership of patients and consumers in EMA working parties**

Membership of working parties (WP)	Members / alternates or observers
Patients and Consumers Working Party (PCWP) + co-chair	19+1 / 16
HealthCare Professionals Working Party (HCPWP)	2
TOTAL	38

### The PCWP and meetings

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 1.5. ).

**Figure 6: The Patients' and Consumers' Working Party (PCWP)**



The list of meetings held in 2015 can be found in Section 1.5. In addition, the PCWP also met on the following occasions:

- 3 June – PCWP plenary meeting (half-day) where they received feedback on topic groups that had been launched in March, an update on participation in EU wide initiatives as well as a presentation on engagement of the US Food and Drug Administration with patients by a visiting fellow from the FDA.
- 25 November - Training session – described further in Section 2.3.
- 26 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. In 2015 an overview of annual EMA activities with patient and consumer involvement was presented along with three patient-led training initiatives. In addition, the results of a survey of interactions of patient with national medicines agencies were described.

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. Topic Groups of the PCWP**

As mentioned in 1.5.1. , topic groups were introduced in 2015 on subjects of mutual interest for EMA and the working parties. 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 addition, eligible organisations are stimulated to participate in the work of the Agency and become engaged in relevant activities for their organisations such as workshops.

The topic groups’ intention is to present concrete recommendations to the working parties based on their objectives. The PCWP has five topic groups, one of which (social media) is a joint group with the HCPWP. The topic groups, listed in Table 3, were launched in March 2015 and are expected, for the most part, to continue in 2016. The key objectives of each topic group are listed below and recommendations from each topic group will be presented to the working parties in 2016. The starting dates and timelines for actions by each topic group are determined by the particular tasks of that group.

**Table 3: PCWP topic groups**

<b>PCWP topic groups</b>	<b>Number</b>
Measure the impact of patient involvement in EMA activities	13
Acknowledge and promote visibility of patient input into the Agency’s activities	18
Training and support for patients involved in EMA activities	13
Involvement of young people in EMA activities	10
Social media	12
<b>Total</b>	<b>66</b>

#### ***Measure the impact of patient involvement in EMA activities***

The Agency has developed a robust system for involving patients, consumers and their representative organisations in its activities including the development of policies, regulatory guidance, and product related evaluation.

This annual report on interactions is presented to the EMA Management Board, to the EMA committees, and subsequently published. The Agency does provide some quantitative and qualitative feedback on

the impact of patient input on particular activities and also includes an analysis of feedback from patients (survey) on their satisfaction as seen within this annual report.

The Agency is frequently asked to further quantify the impact of patient involvement in its activities. There is a need to review the adequacy of the current methodology and determine whether and how it could be improved and/or expanded.

The key objectives include:

- Explore how to measure the benefit/value of patient input on regulatory outcomes
- Explore the impact that involvement in EMA activities has on empowerment of PCOs
- Establish a system for regular cross-Agency collection of quantitative and qualitative data for monitoring and reporting purposes

### ***Acknowledge and promote visibility of patient input into the Agency's activities***

There is a need to raise awareness of the involvement of patients, consumers and their organisations in the work of the EMA and also to further acknowledge the value of their input.

The key objectives include:

- Explore how to raise awareness and visibility of patients/consumers work at the EMA
- Explore how to best acknowledge patient/consumer input in the context of the activities of scientific committees, working parties, scientific advisory groups and other expert groups.

The recommendations will be presented during the meeting with all eligible patient organisations in December 2016.

### ***Training***

To maximise the contribution and experience of patients participating in EMA activities, patients must have an understanding of both the Agency's mandate as well as the expectations of the role they play in the evaluation process.

An EMA training programme, based on a adapted approach depending on the type of participation of the individuals, is available. It is complemented by personalised and one-to-one support to patients involved in specific activities.

Some organisations and collaborative projects have also developed trainings in order to empower patients to play a recognised advocacy role at European level.

A reflection involving the different actors including the EU network Training Centre could further define a core curriculum and look for synergies of action in order to use training resources (both human and financial) in a more efficient way.

The key objectives include:

- Explore synergies with existing training initiatives
- Discuss and explore further training methods and tools for patients involved in EMA activities (see Section 2.3.).

The recommendations will be presented during the joint meeting in March 2016.

### ***Involvement of young people/children in EMA activities***

The EMA has a long history of involving adult patients in its work and has systems in place for their participation across many activities; however this has not as yet included the involvement of young people.

There are ongoing discussions within the PDCO on the value and feasibility of involving these stakeholders and it has been proposed to establish a young person's network with the PCWP.

The key objectives include:

- Identify existing youth groups within eligible organisations; look to create, within the umbrella of the PCWP, a "young person's advisory network" with young participants
- Identify areas and methodologies for the involvement of young people in EMA/ PDCO activities
- Explore how to raise awareness on the need for more participation in paediatric clinical trials
- Plan 20th anniversary activity at the EMA with young people on 07 October 2015 (see 1.3.1.).

### ***Social media***

The growing trend for patients and healthcare professionals to use social media when searching for and communicating on health-related information raises the importance of the Agency engaging more with these communication channels to ensure easy, consistent and timely access to reliable and understandable information on medicines. The ever-increasing role of information technology in health-related matters, including use of e-health records and databases, and social media by consumers and healthcare professionals, also demands that surveillance methods evolve to consider these developments. In addition, the use of social media by patients to connect and exchange information about their condition, treatment and symptoms represents a wealth of information that needs to be both protected and utilised to serve the community.

As social media is changing the nature and speed of healthcare interaction we would like to stimulate discussion around what are the opportunities and the challenges for medicines development, evaluation, surveillance and information.

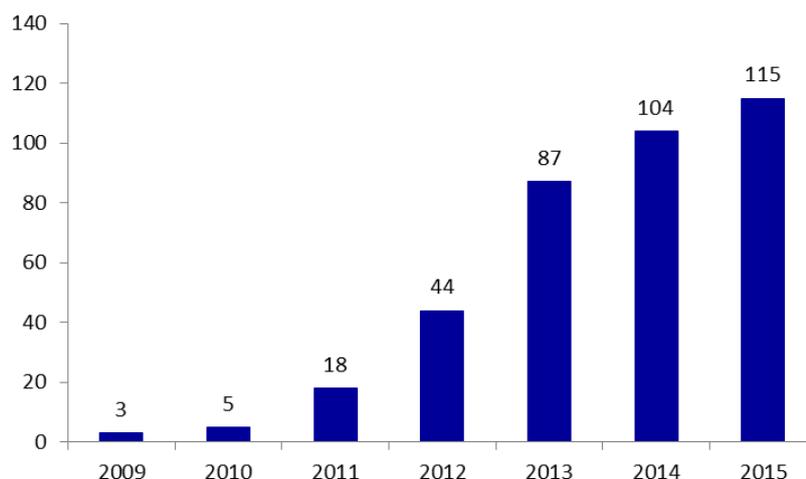
The key objectives include:

- Map current practices in the digital world that are shaping clinical research and clinical care
- Prepare recommendations to EMA and to patients', consumers' and healthcare professional organisations intended to raise awareness of how data and information related with real use of medicines is being collected and used for research and/or other purposes and call for actions as appropriate
- Prepare recommendations to EMA and to patients', consumers' and healthcare professional organisations on how to use their communication channels (internet and social media) more widely, to ensure easy, consistent and timely access to authoritative, reliable and understandable information on medicines
- Identify topics and speakers for a PCWP/HCPWP workshop on social media to be organised in 2016

#### ***2.2.2.3. 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 1.5.

**Figure 7: Number of patients and consumers included in workshops at EMA (2009-2015)**



**2.2.2.4. Overview of activities involving patients' and consumers' organisations in 2015 as representatives of their organisations**

Table 4 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 4 have been described in more detail above. For more information on other activities, please consult the [EMA website](#).

**Table 4: EMA Activities involving patient and consumer organisations**

Activities involving patients' and consumers' organisation	Number of representatives
Ad-hoc observers/experts attending PCWP meetings	25
Observer at HCPWP meetings	1
Departmental meetings with committee representatives	8
Eligible organisations attending annual meeting	13
PCWP/HCPWP social media topic group survey	23
Patient participation in workshops at European Medicines Agency	99
Working group for the preparation of shortages workshop	2
Cross-Committee Task Force on patients registries (4 teleconferences/ meeting)	14
Final PROTECT Symposium: Monitoring benefits and risks of medicines: PROTECT results and recommendations	3
EMA Perception Survey	47
EU Clinical trial portal and union database stakeholders meetings (4 meetings)	17
Clinical trial Regulation programme subgroup 5 - public view (teleconference)	2
PCWP involvement in preparation of Guidelines on the summary of clinical trial results for laypersons	5
PRAC consultation: risk minimisation strategies for medication errors with high strength and fixed combination insulins	1
EMA 20 <sup>th</sup> anniversary event - Orphan medicines – an unaffordable public good?	1
7th Enpr-EMA Workshop - Enpr-EMA - meetings (Promoting high quality scientific research in paediatric medicines)	1
EMA 20th anniversary event - The view from the sharp end: what patients and healthcare professionals can do for us	1

Activities involving patients' and consumers' organisation	Number of representatives
Collaboration between researchers and the EMA on challenges in development of medicines for neonates (Enpr-EMA - WG7)	1
ENCePP - Steering Group meeting	1
ENCePP plenary	1
Meetings with Myeloma UK elicitation study	3
Teleconference with Spinal Muscular Atrophy (SMA) Europe	1
EMA consultation: excipients in the label and package leaflet of medicinal products for human use (Sodium Lauryl Sulfate; Fragrances)	2
Consultation on the publication of the PDCO public summaries of the evaluation of PIPs	1
Science, Medicines, Health: Patients at the heart of future innovation conference	15
EU Network Strategy to 2020 - Written comments	5
<b>TOTAL</b>	<b>293</b>

#### 2.2.2.4.1. Duchenne Muscular Dystrophy meeting – hosted by EMA

The European Medicine Agency (EMA) hosted a workshop in April on developing exon skipping therapies for Duchenne muscular dystrophy; this was organised by this COST Action with the SCOPE-DMD project.

The workshop was an open forum discussion on the regulatory and translational challenges of developing exon skipping therapies for Duchenne and involved patient representatives, EMA staff, regulators, academics, clinicians and industry representatives.

#### 2.2.3. Patients/consumers as individual experts

When patients and consumers are involved in EMA activities on product-specific issues, they do so as individual experts. Table 5 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 5: EMA activities involving patients and consumers as individual experts**

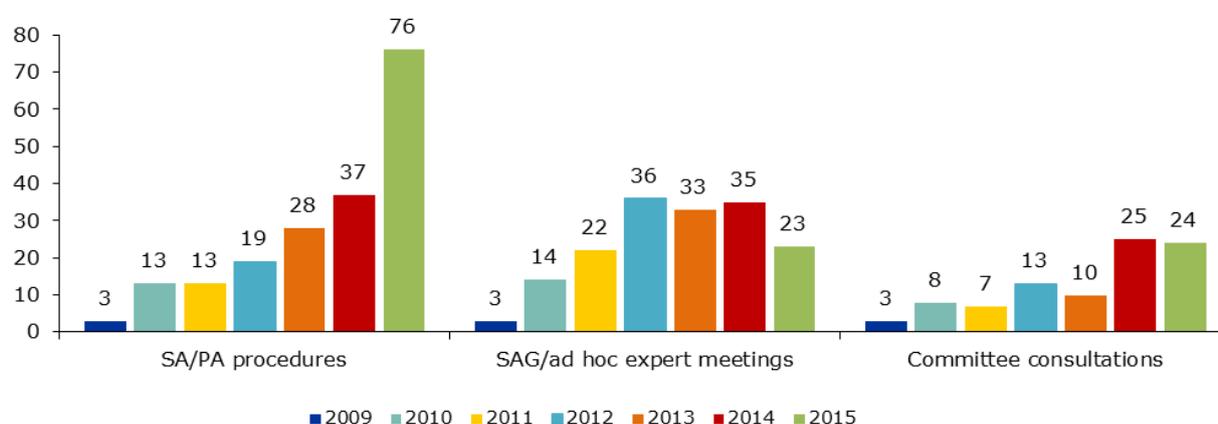
	Activities involving individual experts	Experts
1	CHMP oral explanation – Intuniv	3
2	CHMP oral explanation - Tecfidera	3
3	EMA annual training session	37
4	EPAR summaries - review	47
5	Package leaflets - review	71
6	PRAC / CHMP consultation: Osteonecrosis of the Jaw (ONJ) patient reminder card	6
7	Scientific Advice/Protocol Assistance procedures	76
8	Safety communications - review	19
9	Scientific Advisory Group (SAG)/ad-hoc meetings	23
10	EMA/PRAC consultation: Good Practice Guide on Risk Minimisation and Prevention of Medication Errors – Addendum on Risk minimisation strategies for high strengths and fixed combination insulin products	1
12	CHMP/EMA consultation on expression of strength: medicine for paediatric-onset hypophosphatasia	2

Activities involving individual experts		Experts
13	PDCO consultation with patients (PIP) – insulin medicine	7
14	HPV meeting - parents of vaccinated daughters and one patient	8
15	CHMP/Quality Review of Documents group consultation on PL for HIV class labelling - lipodystrophy	2
16	Participation in COMP meeting/consultation - Haemophilia A	2
17	Lunchtime talk and debate: 'Involving young people in the evaluation of medicines for children'	2
18	Presentation to PDCO on participation of young people in medicines evaluation	2
19	Review of text proposed on body fat changes and lactic acidosis with HIV medicines	1
20	Meeting with Spinal Muscular Atrophy group	3
21	Debriefing conference call - parents of HPV vaccinated daughters	10
22	Meeting at EMA with UPPMD – United Parent Project Muscular Dystrophy	5
23	Reflection paper on a proposal to enhance early dialogue to facilitate accelerated assessment of priority medicines (PRIME) - comments	1
<b>TOTAL</b>		<b>331</b>

### 2.2.3.1. Patient and consumer involvement in scientific meetings

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

**Figure 8: Patient and Consumer involvement in EMA activities (2009-2015)**



#### 2.2.3.1.1. 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 in the case of orphan designated medicines, significant benefit. 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 2015, 76 patients were involved in SA/PA procedures, either in writing and/or in a discussion meeting with the company. Two examples involving different types of input by patient experts are described below.

The first concerns a procedure for a rare lysosomal storage disease. An expert from a patients' association representing all lysosomal storage disorders was invited. While this expert was not personally affected by the disease nor was a parent of a patient, they have extensive experience with patients and the group of diseases as a whole. As such they were able to provide precise information concerning the inclusion/exclusion criteria in particular to the age of the patients, which also tied in with the paediatric component of the scientific advice provided. The global view of the disease that this expert brought to the discussion included a clear picture of the needs of these patients in general as well as an understanding of the current treatments available. In this case, the input did not significantly alter the advice provided by the SAWP but confirmed the input provided by the working party while in the presence of an expert representing those affected.

The second procedure involved a parent of a child affected by a neurological disorder. In this case the patient expert was able to provide specific experience of living with the disease. This expert contributed specifically regarding the endpoints proposed by the sponsor regarding respiratory events.

*"I have found this experience extremely interesting and enriching and I am very honoured to have been able to take part"*  
Patient involved in Scientific Advice

While agreeing with the endpoint proposed, the patient emphasised the importance of this measure in daily life. This issue was addressed at the discussion meeting where the Applicant accepted that an effect on the primary endpoint should be supported by another measure that assesses clinical benefit in a broader way. In addition,

some of the secondary endpoints to be measured (e.g. oxygen saturation) may also help establishing the consequences of an improvement in the primary outcome.

These examples demonstrate that patient input provides not only expertise on specific questions but can also provide general information on living with the disease, its management and impact on daily life. As one member of the EMA Scientific Advice team put it, simply discussing medicines with patients for whom they are destined, contributes a different dimension.

#### **2.2.3.1.2. Input into SAG/ad hoc expert meetings**

The Agency's Committee for Human Medicinal Products (CHMP) and the Pharmacovigilance and Risk Assessment Committee (PRAC) are supported by Scientific Advisory Groups (SAGs) and ad hoc expert groups to provide advice in connection with the evaluation of specific types of medicines or treatments. They consist of European experts selected according to the particular expertise required on the basis of nominations from the committees or the Agency 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 and carers were able to provide valuable and relevant input based on their first-hand experience and knowledge of the disorder.

##### **Intuniv (guanfacine)**

An ad hoc expert group meeting was convened in the context of the assessment of the Intuniv (guanfacine) application for marketing authorisation for attention deficit hyperactivity disorder (ADHD) as requested by the CHMP during the March 2015 plenary meeting.

In addition to ADHD clinical experts, rapporteurs and assessors, and EMA staff, three patients' representatives from different EU countries also participated in the meeting.

## Tecfidera (dimethyl fumarate)

A Scientific Advisory Group (SAG) was convened in June regarding risk factors for developing progressive multifocal leukoencephalopathy (PML) in patients treated with the multiple sclerosis medicine Tecfidera (dimethyl fumarate). Three patient representatives were invited to attend the SAG meeting along with clinical experts, rapporteurs and assessors and EMA staff.

### 2.2.3.1.3. 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 6.

**Table 6: Committee/Working party consultations with patient organisations**

Committee	Subject	Contribution of patients/consumers
PRAC / CHMP	Consultation on Bisphosphonates and denosumab containing medicinal products - risk of osteonecrosis of the jaw (ONJ) - patient reminder card	The PRAC sought the input of patient representatives on the proposed educational material – specifically a patient reminder card. Input was provided by the patients on whether the risk of ONJ was adequately explained, including the population at higher risk as well as whether the information was clear and comprehensible.
EMA/PRAC	Following approval of higher strength insulins, concerns about medication errors associated were raised by the PRAC. To address the risk of errors in a harmonised way and to avoid significant over- or under-dosing of insulin, a strategy to minimise the potential risk of medication errors was developed on risk minimisation and prevention of medication errors.	Patients were consulted on the draft guidance and communication on risk minimisation strategies for use of high strength and fixed combination insulins and provided their input on the proposed measures.
PDCO	The PDCO requested the input of patients during the evaluation of a medicine indicated in the treatment of type II diabetes mellitus (aged 10 to less than 18 years) consisting of an implanted mini pump able to deliver a continuous subcutaneous dose of insulin. The PDCO wanted feedback on the potential benefit for young people with type II diabetes for this new method of administration, in particular regarding adherence to treatment, local acceptance, and overall acceptability.	Patients provided their views regarding the potential of the implant as a viable option in i) helping ensure that young type II diabetic patients take their medicine as prescribed, ii) the need for such alternatives in this age group as well as iii) the likelihood that these patients would be willing to accept to have this implant.
PDCO	Two patient representatives were involved in discussions with the PDCO regarding the involvement of young people in the evaluation of medicine, prior to the lunch talk described in section 1.3.1.	A young student and two youth leaders gave a presentation to the PDCO regarding their work with young people.
CHMP/QRD	The CHMP/QRD consulted with patients concerning the wording to be included within the package leaflets for HIV class medicines specifically addressing the potential risk of lipodystrophy with these medicines.	The patients consulted were in agreement with the proposed text

Committee	Subject	Contribution of patients/consumers
CHMP/QRD	During the CHMP evaluation of a medicine for paediatric-onset hypophosphatasia consulted with patients / carers to gain their input on the proposed information to be included on the outer packaging, package leaflet and injection guidance, especially regarding the different strengths and presentations available.	Patients gave input on the clarity and comprehensibility of the proposed information, especially bearing in mind that the medicine is usually self-injected (and/or by parent/carer).
CHMP	Several patients were also involved directly within CHMP plenary meetings. See description below for more details of this pilot phase.	Tecfidera and Intuniv
COMP	The COMP invited two patients as external experts to its October plenary meeting to address the question of significant benefit for a medicine for Haemophilia A	Two patients addressed the questions on significant benefit by providing critical information on major contribution to patient care, sharing both their personal experiences as well as the patients they work with.
CAT	A patient expert regularly attended the CAT meetings during 2015 to support the patient member	

### ***Patient involvement at the CHMP***

The CHMP is the committee that is responsible for preparing the Agency's opinions on questions concerning medicines for human use and plays a key role in the marketing procedures for medicines in the European Union.

In the context of the [pilot](#) phase (launched in September 2014) to include patients directly in the benefit-risk evaluation of medicines within the oral explanations at CHMP meetings, patients participated within two such procedures during 2015; Intuniv and Tecfidera.

The patients had previously participated in the Scientific Advisory Group meetings which had been convened by the CHMP to discuss issues in relation to these medicines (for more details see Section 2.2.3.1.2.) and they were subsequently invited to join the CHMP plenary meeting during the oral explanation and discussion.

The CHMP invited patients to the June plenary meeting to their discussion on Intuniv, which was already the subject of a SAG meeting, as described earlier. A commonly reported side effect of Intuniv is sleepiness (or sedation). Due to the nature of ADHD, it is difficult to distinguish between improvement of symptoms and sedation. The patient representatives highlighted their concerns on the risk of sedation and emphasised that sleepiness at different times of day has different consequences.

Patient representatives and physicians were also concerned about the implications on daily activities, as well as potential cognitive impairment when using a sedative drug in children. The group contributed their perspective that the sedation was not very clearly described.

Patients were also invited to join the September CHMP plenary for its discussion on Tecfidera. A SAG had also been held where the risk factors for progressive multifocal leukoencephalopathy (PML) were discussed. PML is a rare brain infection caused by John Cunningham (JC) virus, which is very common and found in the general population and is normally harmless. However, in persons whose immune system is compromised it can lead to PML.

Preventative measures were considered and patients expressed the view that they were more concerned about the loss of efficacy rather than lymphopenia and the potential PML risk as presented by data until now. They supported increased levels of monitoring (e.g. scans) that would help in the monitoring of the risks and in their understanding of the risks and the decisions on potential treatment options.

Overall the members of the CHMP benefitted from hearing directly from the patients and carers their views on the benefits and risks of the medicines under review from the perspective of patients and carers living with the disease being discussed. Their presence also gave an opportunity for CHMP members to ask questions to the patients during the discussions.

This timescale for the pilot (originally one year) has been extended into 2016 to enable more products and patients to be included, and allow for a more robust analysis to be performed at the close of the pilot.

### 2.2.3.2. 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).

In Figure 9, the number of documents reviewed by patients and consumers is shown.

**Figure 9: Documents reviewed (2007-2015): Package leaflets and EPAR summaries and Safety communications**

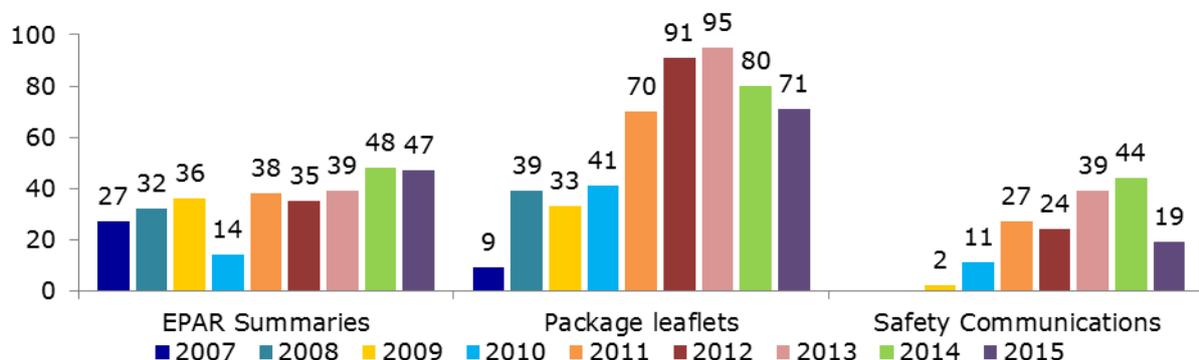
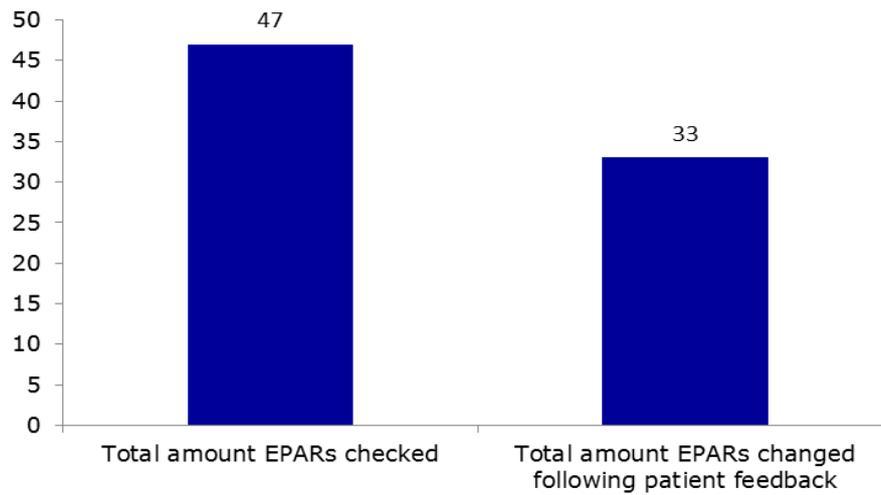


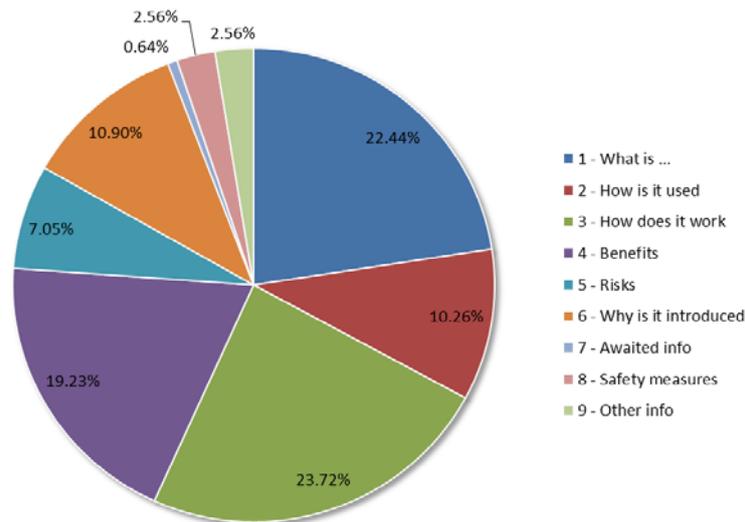
Figure 10 shows the total number of EPARS reviewed by patients and the total number that were changed as a result of patient feedback. Of the 47 EPARS reviewed, 33 (70%) were altered as a result of patient input.

**Figure 10: Total EPARS reviewed by patients and changes made to document**



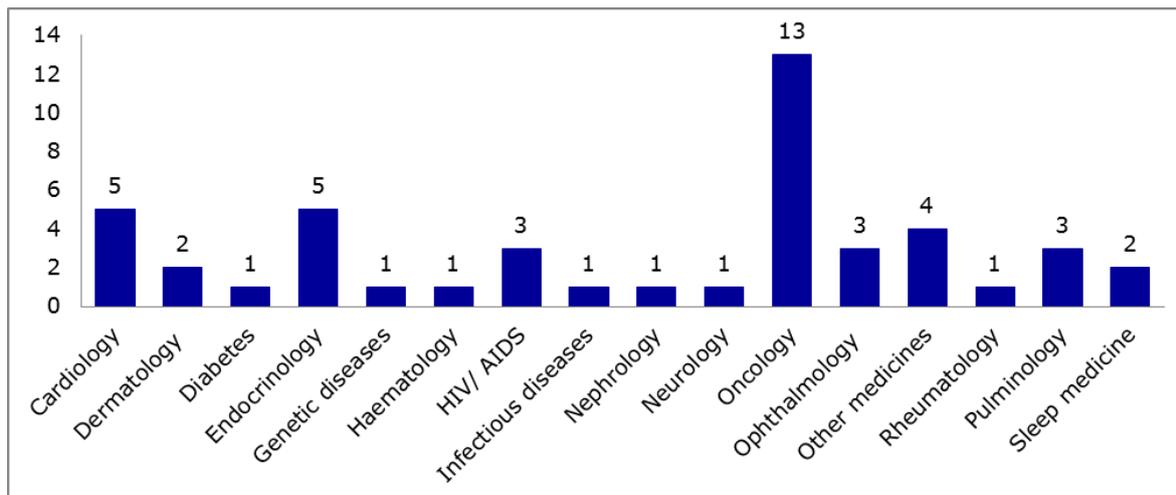
In Figure 11, the distribution of the comments received per section of the EPAR is shown, not surprisingly the majority of comments are in the sections relating to ‘What is the medicine’ and ‘How does it work’ as well as the ‘Benefits demonstrated in the studies’.

**Figure 11: Distribution of comments by patients in EPAR summaries per section**



In Figure 12, we see the numerous therapeutic areas that are covered by the EPAR summaries reviewed by patients.

**Figure 12: Therapeutic areas covered by EPAR summaries in 2015**



### **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.

**Webpages:** The EMA website has a wealth of information, however finding this information is not always easy. Information specific for patients and consumers is highlighted in a dedicated page entitled Training and Support and one objective outlined in the revised framework for interaction is improve capacity building and awareness raising activities.

One objective of the Training topic group (described in section 2.2.2.2.) is to review the focus of these pages to ensure they are tailored to the needs of patient and consumers. In this way, the most relevant and beneficial information will be made even more visible and useful videos and materials will be clearly indicated.

**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:**

Based on feedback from previous participants and an internal recognition of a need to move towards a more interactive hands-on format for annual training day, a new format was introduced in November 2015. As patients are involved all along the lifecycle of a medicine, minimal presentations and breakout sessions were used to illustrate the role of patients and the expectations of the Agency for various activities from involvement in pre-submission and evaluation phases to post-authorisation.

Participants were introduced to the work of the Agency and the role of patients in a formal presentation of two parts, each presentation was separated by break-out sessions using examples of where individual patient expert input is sought in EMA activities. These 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. Each breakout group consisted of

approximately 10 participants and was facilitated by a member of EMA staff from the specific department/division responsible for that activity.

While EMA colleagues have always been involved in the Training day, attending and presenting on various topics, the new format provided both the facilitators and the participants much more contact and opportunity for exchange on the subject of the activity but also more broadly. Positive feedback was received in the follow up survey.

## **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 7.

**Table 7: EMA participation in external patients' and consumers' meetings**

<b>Organiser/Event</b>	
1	Melanoma Patient Network Europe (MPNE) Conference: the risk of not taking risks in melanoma
2	European AIDS Treatment Group (EATG): stakeholders meeting
3	CHMP and CAT Strategic review and learning meeting
4	EUPATI Project Advisory Board meeting
5	PROTECT Final Symposium
6	EURORDIS rare disease day media event
7	EFPIA 50 year anniversary of EU pharmaceutical legislation
8	Newcastle University – Workshop: Participants not subjects – engaging patients and families in paediatric clinical research
9	MPNE 2015 conference
10	University of Copenhagen: Patient Involvement in medicines development and approvals
11	ISPOR 4th Patient Representatives Roundtable - Milan
12	Workshop on stem cell therapies and gene therapies (Genetic Alliance UK/Wellcome Trust - Medical Research Council Cambridge Stem Cell Institute)
13	FT global pharmaceutical and biotechnology conference
<b>Promoting patient engagement</b>	
14	DIA Euromeeting
15	CIRS patient Engagement workshop
16	EUnetHTA-EMA meeting
17	Giving patients a voice-Federal Joint Committee
<b>Training</b>	
18	EURORDIS Summer School – training for patients
19	London School of Economics (LSE) and European federation of Neurological Associations (EFNA) - Pharmaceutical Policy, Pricing, and Reimbursement: A course for Patient Advocates
20	EUPATI training course for patients – Barcelona

## 2.5. Organisations involved in EMA activities during 2015

There were no changes in the list of EMA eligible organisations in 2015. The 36 patients' and consumers' organisations are shown in Table 8 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.).

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 8: 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	EMA eligible organisations
5	European AIDS Treatment Group (EATG)
6	European Cancer Patient Coalition (ECPC)
7	European Federation of Allergy and Airways Diseases Patients' Associations (EFA)
8	European Federation of Neurological Associations (EFNA)
9	European Foundation for the Care of Newborn Infants (EFCNI)
10	European Gaucher Alliance (EGA)
11	European Genetic Alliances' Network (EGAN)
12	European Haemophilia Consortium (EHC)
13	European Headache Alliance (EHA)
14	European Heart Network (EHN)
15	European Institute of Women's Health (EIWH)
16	European Liver Patient Association (ELPA)
17	European Multiple Sclerosis Platform (EMSP)
18	European Network of Fibromyalgia Associations (ENFA)
19	European Organisation for Rare Diseases (EURORDIS)
20	European Parkinson's Disease Association (EPDA)
21	European Patients' Forum (EPF)
22	European Prostate Cancer Coalition (EUomo)
23	European Public Health Alliance (EPHA)
24	Fabry International Network (FIN)
25	Global Alliance for Mental Illness Advocacy Networks (GAMIAN-Europe)
26	Health Action International (HAI)

EMA eligible organisations	
27	Insulin Dependent Diabetes Trust (IDDT)
28	International Alliance of Patients' Organizations (IAPO)
29	International Bureau of Epilepsy (IBE)
30	International Diabetes Federation European Region (IDF Europe)
31	International Patient Organisation for Primary Immunodeficiencies (IPOPI)
32	Myeloma Patients Europe (MPE)
33	Pain Alliance Europe (PAE)
34	Spinal Muscular Atrophy Europe (SMAE)
35	Thalassaemia International Federation (TIF)
36	The European Consumers' Organisation (BEUC)

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 2015, in addition to the 36 eligible organisations (Table 8), another 45 patients' and consumers' organisations also interacted with the Agency and are listed in Table 9.

**Table 9: List of organisations consulted by EMA on specific areas**

Organisations consulted by the EMA on specific areas	
1	Action Duchenne
2	ADHD-Europe
3	Association Francaise Contre les Myopathies (AFM)
4	Arbeitskreisleiter Chorioideremie der Pro Retina Deutschland e.V.
5	Cystic Fibrosis Trust
6	Duchenne Alliance
7	Duchenne Children's fund
8	Duchenne Ireland
9	Duchenne Parent Project
10	Duchenne Parent Project Belgium
11	Duchenne Parent Project les myopathies
12	Duchenne Parent Project Netherlands
13	Duchenne Parent Project Spain
14	Duchenne Parent Project Romania
15	Dutch Celiac Society
16	EuropaDonna
17	European Federation of Crohn's and Ulcerative Colitis Associations (EFCCA)
18	Foundation of European Nurses in Diabetes (FEND)
19	Gaucher Association
20	Guildford & South West Surrey Diabetes UK Group
21	Hungary-IDB
22	Institut de Myologie
23	Irish Haemophilia Society Ltd.
24	Klub nemocnych cystickou fibrózou, o.s.

Organisations consulted by the EMA on specific areas	
25	Melanoma Patient Network Europe
26	Myeloma Euronet
27	Myeloma UK
28	Muscular Dystrophy UK
29	MPS Society
30	National organisation of sjogren patients
31	National Rheumatoid Arthritis Society (NRAS)
32	NHS Foundation Trust
33	PHA Europe
34	Portuguese Association of Young Diabetics
35	ProRetina Germany
36	Retina Europe
37	Rett Syndrome Europe
38	ScotCRN Young Person's Advisory Group
39	Spinal Muscular Atrophy Support UK
40	Tampere Diabetes Association (Finland)
41	The Society for Mucopolysaccharide Diseases
42	UK Thalassaemia Society
43	United Parent Projects Muscular Dystrophy
44	Vereniging voor Kinderen met Stofwisselingsziekten
45	Vaccinationsforum

## **2.6. Involvement in Networks and research projects**

### **2.6.1. 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.

### **2.6.2. Elicitation of Patient Preferences and Values on Benefits and Risks project**

There has been increased interest in exploring ways to elicit patient preferences about the benefits and risks of medicines. Although patient preferences are not expected to replace regulator's expert judgments, they can be informative because they can provide support for regulator's expert judgments or otherwise highlight situations where regulator's and patients' preference might diverge and where there is greater need for communication about the regulators' decisions.

There is no consensus on what are the best methods to elicit patient preferences (value judgments and trade-offs) about benefits and risks of medicines.

One way to include the perspective of the patient is to invite one or more patients to participate in committee discussions, etc., where they can inform the committee members about their experience with the disease and how this affects their view on the product's benefit-risk balance. Although such

oral discussions may provide some insight into how the participating patients value the product's favourable and unfavourable effects, due to time constraints it will only be feasible to hear a very small number of patients (e.g., two or three individuals). The generalisability of the information obtained during these sessions will therefore be limited.

A more comprehensive approach to capturing patient preferences would be to conduct a survey in a large group of patients (e.g., 200-300) and then use statistical analysis to produce summary measures that can be presented to the regulatory committee. Although this approach is likely to provide a much better picture of how patient preferences are distributed across the target population, the feasibility and effectiveness of this approach is still unclear and needs to be explored.

In 2015 the EMA piloted one preference elicitation technique with two patient groups: Melanoma Patients Europe and Myeloma Patients Europe. The purpose was to explore the feasibility of using a multi-criteria decision analysis (MCDA) survey technique to elicit preferences on the benefits and risks of treatment. While involving only small numbers the results were promising and were [published](#). The intention is to further these studies using a larger number of patients.

### **2.6.3. ADAPT-SMART**

The EMA is leading the IMI-funded project [ADAPT-SMART](#) (Accelerated Development of Appropriate Patient Therapies - a Sustainable, Multi-stakeholder Approach from Research to Treatment-outcomes), whose key objective is to provide patients with more appropriate access to innovative medicines.

The project duration is 30-month and the specific aim is to establish collaborative solutions to foster the development of Medicines Adaptive Pathways to Patients (MAPPs) in Europe, encouraging more efficient ways of developing and regulating medicines. MAPPs seek to foster access to beneficial treatments for the right patient groups at the earliest appropriate time in the product life span in a sustainable manner.

In the [kick-off meeting](#) in September, approximately 90 representatives from patient organisations, health-technology-assessment bodies, regulators, payers, academia and industry attended.

## ***2.7. Exchange of practices of patient engagement***

### **2.7.1. Involvement of patients and consumers at a Member State level: exchange of best practices within the EU Regulatory Network**

Continuing in the spirit of nurturing dialogue between stakeholders in medicines development and the National Competent Authorities (NCA), a survey was prepared to gain an understanding of their existing interactions with patient and consumer organisations.

The [survey](#) was drafted in conjunction with the PCWP and shared with the Working Group of Communications Professionals of the Heads of Medicines Agencies (HMA) prior to its finalisation and dissemination. The scope of the questions included whether the Agency involved patients in its activities, the types of activities, existence of any requirements prior to interactions taking place, reimbursement for participation, training provided as well as what they would consider success or challenges of interacting with these groups.

Responses were received from 15 countries; a majority stated that they did work with patients. These interactions were initiated upon the request of both patients and the Agency and primarily disease-specific groups were involved. Individual patients, umbrella organisations and consumer groups were also involved with the national agencies. There was a diversity of the types of activities that patients

were involved in and were primarily on a case by case basis, followed by general updates on medicines, dissemination of material, awareness campaigns and members of consultative committees.

In order to work with the Agencies, a declaration of interest and confidentiality were frequently required as well as official accreditation of the association. Of the Agencies that involved patients, the majority felt that it had been beneficial to involve patients and that a better understanding of regulation on the side of the patients would be helpful but that both sides needed better infrastructure and resources to support these interactions. It was largely felt that these interactions have been assisted by mutual trust and ongoing communication.

Different agencies felt that they were at different stages in their interactions with patients and even those that did not currently involve patient did have some contact with them such as disseminating information to them.

In a follow up to the four Agencies that presented to the PCWP in 2014 regarding their interactions with patients, a representative from the Swedish medicines agency (MPA) described an update of their proposals for patient engagement and was accompanied by a representative from the Swedish HTA agency (TLV).

### **2.7.2. Fellowship exchanges with US Food and Drug Administration (FDA)**

A mutual exchange of staff responsible for regulatory activities involving patients occurred between the US Food and Drug Administration (FDA) and the European Medicines Agency (EMA). One staff member from each Agency spent two weeks in the other agency in order to gain an in-depth overview of patient engagement.

An EMA staff member spent two weeks at the FDA in December 2014 and an FDA staff member reciprocated with two weeks at EMA in June 2015. Both concluded that overall the fellowship was a very interesting, rewarding and worthwhile experience.

The objectives of the fellowship included gaining an overall understanding of how FDA engages with patients, including practicalities such as how and when patients collaborate with the FDA, how they are selected and screened for potential conflicts and what training and support is provided to optimise participation.

The results of this exchange will benchmark practices and policies and identify areas that could benefit from an exchange of EMA/FDA experience. In addition, a platform has been established for FDA/EMA collaboration on patient involvement, this will result in the establishment an EMA-FDA cluster (platform for regular exchange of information on topics of mutual interest).

Finally, this provided a good opportunity for EMA to learn from FDA's experience of holding "public meetings".

### **2.8. Next steps**

In 2016, the Agency will continue to focus on the following areas:

- Establishing a pool of experts
- Developing capacities
- Raising awareness
- Implementation of public hearings

### 3. Interaction with healthcare professionals

#### 3.1. Introduction

During 2015, the Agency continued to engage with healthcare professionals to facilitate the inclusion of clinical practice input perspective in EMA activities aimed at supporting medicines' development, evaluation and continuous improvement of the pharmacovigilance system.

Efforts were directed to maintaining the network of healthcare professional organisations and supporting them in the transition to the new transparency requirements, as already described in section 1.2. As reflected in Figure 13, 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 2015, these 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.

As in previous years, the Healthcare Professionals' Working Party (HCPWP) served as a platform to promote a better understanding of the Agency's activities and involvement in EU-wide initiatives, joining efforts with its counterpart – the Patient and Consumers' Working Party (PCWP). More specifically, the HCPWP expanded discussions through the creation of dedicated topic groups (as described in 1.5.1. and 2.2.2.2. ), enriched the brainstorming phase of the EMA initiative to develop a more structured collaboration with Academia and initiated a reflection on the need to review the framework for interaction between the Agency and healthcare professionals

As foreseen in the 2014 report, the Agency continued its efforts, in close collaboration with the representative organisations, to expand outreach to general practitioners and reflect on how interaction with this particular group of healthcare professionals may be improved in the future (as detailed in 3.1.3).

**Figure 13: Regular interaction between the Agency and the network of European healthcare professional organisations**

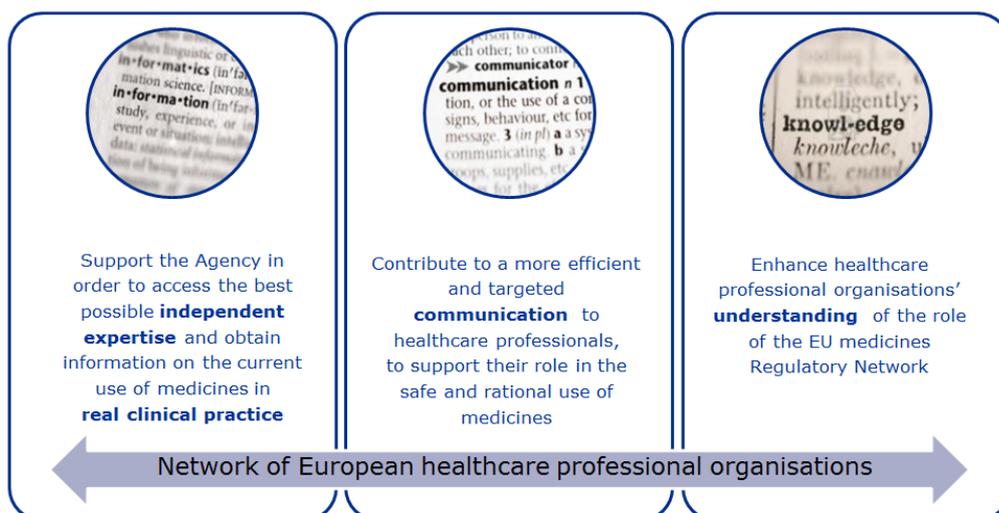
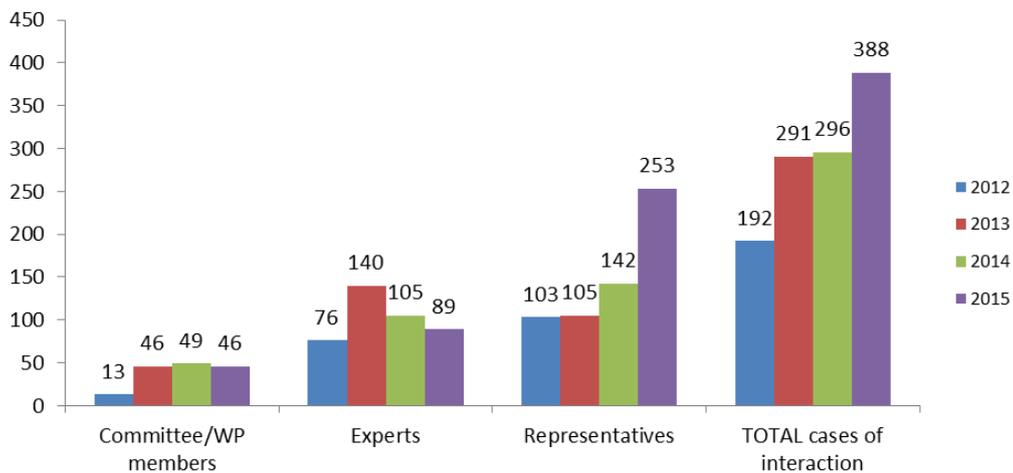


Figure 14 provides an overview of the sustained involvement of healthcare professionals in EMA core activities, which will be further elaborated on in the following sections.

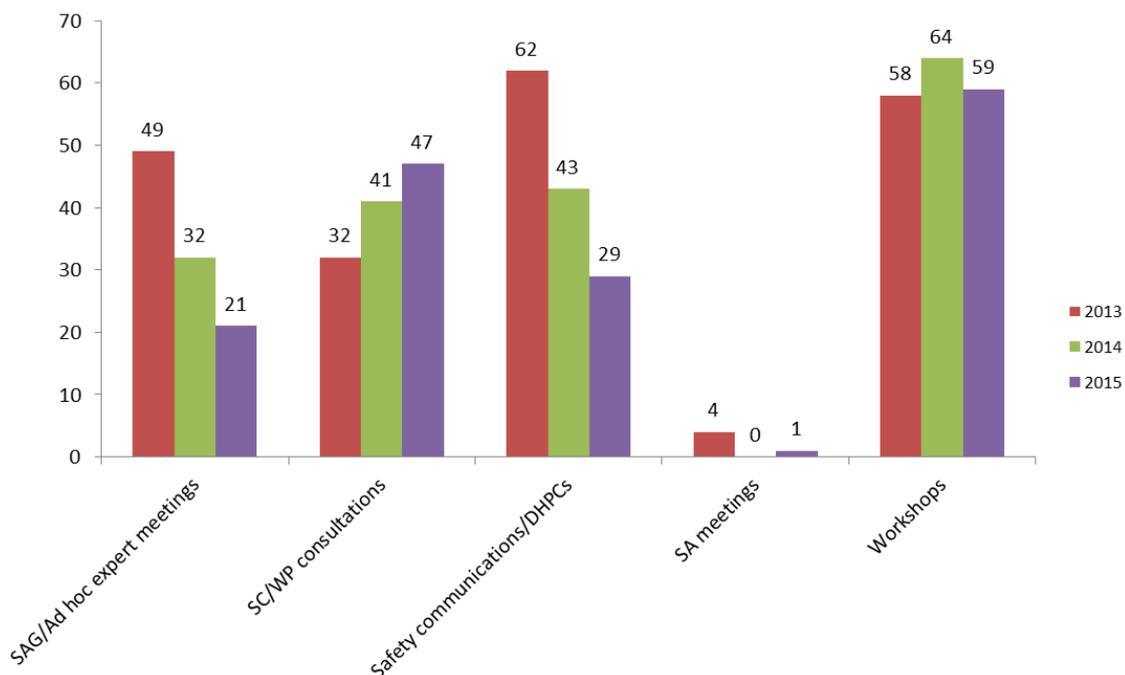
**Figure 14: Involvement of healthcare professionals as Committee/ Working Party members, experts and representatives of organisations**



A marked increase in involvement of representatives in 2015 compared to previous years is seen. This is in part due to the membership of the HCPWP topic groups, as mentioned in section 1.5.1. , as well as respondents to the surveys performed throughout the year. Most topic groups are of a temporary nature and surveys are carried out on a need-identified basis. Although there seems to be an overall increase of cases of interactions over the years, these 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 Figure 15 the involvement of healthcare professionals in the Agency’s scientific activities and workshops is illustrated.

**Figure 15: Involvement of healthcare professionals in EMA activities (2013-2015)**



As seen in Figure 15, there were fewer requests in 2015 for SAG/ ad-hoc expert meetings and a lower amount of safety communications and DHPCs to be reviewed. These lower numbers are due to variations in the number of requests linked to products undergoing assessment at the EMA. At the

same time scientific committee and working party consultations have shown a gradual increase over time.

### 3.2. 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 16 shows the different activities associated with these different types of representation.

**Figure 16: Healthcare professionals in EMA activities and scope of representation**



#### 3.2.1. Healthcare professionals representing *healthcare professionals’* organisations

##### 3.2.1.1. Membership in EMA management board and scientific committees

As described in Figure 16, 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.

In addition, healthcare professionals are represented in three of the six human scientific committees at the EMA (See Table 10). 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 10: Membership of healthcare professionals in EMA management board and scientific committees**

EMA Management Board and Scientific Committees	Members / alternates
------------------------------------------------	----------------------

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

### ***Interactions with healthcare professionals' organisations representatives from the EMA scientific committees***

In 2015, the Department of Patients and Healthcare Professionals initiated frequent and regular meetings throughout the year with the representatives of healthcare professionals' organisations who are members of the scientific committees of the EMA. The purpose of the meetings is to provide support to these members where needed, be available for any clarifications as well as to assist in the identification of specialised healthcare professionals for committee consultations.

For the patients as members of the EMA scientific committees, their role has been described in a document elaborated in 2009. Current plans exist to update and adapt this document to include the role of healthcare professionals as well and to highlight the added value of these members to the committees and to the work of the Agency.

## **3.2.2. Healthcare professionals representing *their* organisations**

### ***3.2.2.1. 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 18 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 11). 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 reciprocal observers who follow the work of the working party and present their particular perspective where necessary.

**Table 11: Membership of working parties**

Membership of working parties (WP)	Members / alternates or observers
HealthCare Professionals Working Party (HCPWP) + co-chair	18 + 1 / 13
Patients and Consumers Working Party (PCWP)	1
<b>TOTAL</b>	<b>33</b>

*'The implementation of topic groups composed of EMA and the Healthcare Professionals Working Party (HCPWP) and the Patients and Consumers Working Party (PCWP) has proven to have a valuable and positive impact in the interaction and collaboration between both working parties (WP). This improved interaction translates into an enhanced participation of members of both WPs and the establishment of mixed working groups focusing on specific topics of interest for both patients and HCPs. It is hoped that this is the way to deepen in the coming years, aiming to improve the collaboration and mutual understanding between patients and healthcare professionals and their contribution to the activities of the EMA.'*

*(Gonzalo Calvo, HCPWP co-chair)*



The HCPWP met three times in 2015. These meetings were mostly joint with the PCWP where a variety of topics were presented and discussed ranging from updates on EU legislation to different EMA core initiatives and projects to activities started by eligible organisations themselves. See section 1.5. for a comprehensive overview.

In addition to the joint meetings with the PCWP, the HCPWP had a half-day [meeting](#) mostly dedicated to the EMA framework of collaboration with academia, to gather the views of the learned societies represented within the HCPWP. To stimulate discussion, results of interviews and a survey conducted within EMA to gather information on current interactions with academia regarding different EMA activities were shared. During the discussion and brainstorm session the working party members were asked to give input on key elements that should underpin the relationship between learned societies and regulators and where opportunities for collaborations can be identified. The HCPWP members were overall positive about developing a more structured collaboration between the EMA and academia and suggested that the internal exercise carried out by the Agency to identify the unmet needs, gaps and opportunities should be also carried out amongst learned societies. Furthermore they commented on the necessity to identify and target specific audiences within academia to enable meaningful collaborative approaches, use existing platforms and channels to integrate and foster knowledge about the EMA and the regulatory environment and use them for targeted communication in a strategic way. The comments gathered during the meeting will inform the drafting of the framework. More detailed information on EMA's initiative to have a formal framework can be found in section 3.2.2.

**Figure 17: The HealthCare Professionals' Working Party (HCPWP)**



### 3.2.2.2. Topic groups of HCPWP

As introduced in section 1.5.1. , dedicated groups were created to cover topics of mutual interest for EMA and the working parties. The aim of the topic groups is to enable brainstorming in smaller groups between plenary working party meetings, promote further discussion on specific topics and allow better utilisation of time during the face-to-face working parties' meetings. Additionally it stimulates eligible organisations to participate in the work of the Agency and engages them in organising activities relevant for the organisations such as workshops. The topic groups' intention is to present concrete recommendations to the working parties, based on their key objectives, in 2016. The HCPWP has four topic groups; "social media" (in association with the PCWP), "risk minimisation measures and assessment of their effectiveness", "information on medicines" and "academia, learned societies and healthcare professionals' organisations". The topic groups started between June and October 2015, with different levels of progress made at the end of 2015. All topic groups are expected to continue with their work in 2016 and the presentation of recommendations is planned for the second half of that year. An overview of the cases of interaction emerging from the topic groups in 2015 can be seen in Table 12.

**Table 12: HCPWP topic group activities**

HCPWP topic group activities	Number
Topic group on EMA-CHMP-PRAC projects on information on medicines (teleconferences)	12
Topic Group on Risk minimisation measures and assessment of their effectiveness	3
Topic group on Academia, learned societies and healthcare professional organisations (teleconference)	6
Topic group on Social Media (teleconferences and meeting)	25
<b>TOTAL</b>	<b>46</b>

#### **Social media**

The growing trend for patients and healthcare professionals is to use social media when searching for and communicating on health-related information. This raises the importance of the Agency engaging more with these communication channels to ensure easy, consistent and timely access to reliable and understandable information on medicines. The ever-increasing role of information technology in health-related matters, including use of e-health records and databases, and social media by consumers and healthcare professionals, also demands that surveillance methods evolve to consider these developments.

As social media is changing the nature and speed of healthcare interaction the topic group aims to stimulate discussion around what are the opportunities and the challenges for medicines development, evaluation, surveillance and information.

The social media topic group is a joint topic group with members from organisations represented in the PCWP or the HCPWP. The key objectives include:

- Map current practices in the digital world that are shaping clinical research and clinical care
- Prepare recommendations to EMA and to patients', consumers' and healthcare professional organisations intended to raise awareness of how data and information related with real use of medicines is being collected and used for research and/or other purposes and call for actions as appropriate
- Prepare recommendations to EMA and to patients', consumers' and healthcare professional organisations on how to use their communication channels (internet and social media) more

widely, to ensure easy, consistent and timely access to authoritative, reliable and understandable information on medicines

- Identify topics and speakers for a PCWP/HCPWP workshop on social media to be organised in 2016

The topic group had their first meeting in June 2015 and had regular feedback moments throughout the second half of the year. One of their achievements was to hold a scoping survey amongst all eligible organisations to better understand the organisations' social media usage. This survey raised questions on more concrete experiences and reasoning behind organisations' usage and a follow-up is expected in 2016. Additionally, the topic group members gained a better understanding of EMA's use of social media and its work to develop a social media strategy as well as learned about the ongoing IMI project WEB-RADR. Based on discussions held so far within the topic group, the proposal came to consider the topic group as an ongoing group and broaden the scope of its discussions to encompass digital media and health.

### ***Risk minimisation measures and assessment of their effectiveness***

Planning and implementing risk minimisation measures and assessing their effectiveness are key elements of risk management. A variety of tools are currently available for additional risk minimisation and this field is continuously developing, with new tools likely to be developed in the future building upon advances in technology. In addition, the evaluation of effectiveness of risk minimisation measures is an evolving area of medical sciences with a need for universally agreed standards and approaches.

Whilst taking advantage of relevant elements of methodology from pharmacoepidemiology and other disciplines such as social/behavioural sciences and qualitative research methods, it is important to bring on board healthcare professionals (HCPs) and patients in the shaping of adequate and proportional risk minimisation measures, which are balanced with the benefit for patients and produce the desired public health outcome in the context of the healthcare delivery system. The group focusing on this topic is composed by HCPWP members and relevant EMA staff. The group aims to also have members from PRAC to support linkage to this committee. The key objectives include:

- Discuss current practices/experience (regulator and HCP perspectives ) in the development and implementation of additional risk minimisation measures, using concrete examples of risk minimisation tools;
- In the context of the PRAC activities, brainstorm on how to facilitate input from HCPs into the feasibility, information and evaluation of risk minimisation measures; explore aspects around product-specific issues, therapeutic class and overall therapeutic environment and prepare recommendations as appropriate;
- Discuss how to better inform HCPs about ongoing activities and initiatives within the EU regulatory network related with post-authorisations Efficacy and Safety studies, registries, medication errors, RMP summaries and safety communications and prepare recommendations as appropriate.

The topic group started in October 2015 following the PCWP/ HCPWP workshop on risk minimisation measures and assessment of their effectiveness organised in September 2015 (for information on this workshop see section 1.6.5. ). To advance discussions, the group agreed to look at a set of examples of medicinal products for which (additional) risk minimisation measures were set up and analyse what worked well and whether there were elements of it that could have been done differently to optimise the final outcome. The group is expected to recommend a set of criteria to be put to the consideration of PRAC for HCPs' involvement in RMM development and implementation.

### **EMA/ CHMP/ PRAC projects on information on medicines**

Challenges posed by increasing data and scientific knowledge, unavoidable uncertainties, demand for more information including for individualised therapy, request for easily accessible information, and different needs and practices raise the importance of maintaining high quality information throughout the lifecycle of the medicine, ensuring it is consistently up-to-date and meets the needs of the users.

They also raise the need to ensure that product information (Summary of Product Characteristics (SmPC), package leaflet and labelling) is integrated with other information on medicines produced by regulatory bodies and is considered in the wider context of information sources, information targets and information seekers. For example, some of the areas that would benefit from additional discussion include: a) how benefit-risk information in assessment reports and quality assurance of SmPCs could best respond to healthcare professionals' information needs; b) how to promote consistency between SmPC and therapeutic guidelines/ prescribing recommendations; c) interaction with drug bulletins.

The topic group on information on medicines is a joint initiative between the HCPWP, EMA, CHMP and PRAC. The key objectives include:

- Setting the scene and summarising identified challenges
- Discuss the target audience(s) of the different information on medicines produced by EMA (e.g. healthcare professionals, those treating patients, bodies preparing therapeutic guidelines, or, journals/drug bulletins/other information providers)
- Discuss healthcare professional organisations' role in the information chain, e.g. for communicating regulatory information or therapeutic guidelines/prescribing recommendations
- Identify ways to facilitate input from healthcare professionals into the preparation and update of regulatory information
- Prepare recommendations to EMA and to healthcare professional organisations on:
  - a) how to use available resources to maintain high quality of product information throughout the lifecycle of the medicine whilst ensuring it reflects as much as possible clinical practice reality (with proposals for concrete pilots);
  - b) how to use or improve current EMA information outputs to support clinical practice;
  - c) how to bridge regulatory outputs with therapeutic guidelines/prescribing recommendations.

The topic group started its work in July 2015 by discussing the extent and variety of audiences, needs and documents as well as the large diversity regarding medical practice and access to information between countries in Europe. The group then recommended carrying out a survey within Healthcare Professionals to clarify which information they use and for which purpose. The survey was prepared at the end of 2015 and expected to be launched and analysed in Q1 of 2016.

### **Academia, learned societies and healthcare professionals' organisations**

EMA interactions involving healthcare professionals range from information and consultation to participation in the scientific activities of the Agency and its committees, and review of information intended for the public. In December 2011, the Agency's Management Board endorsed a framework of interaction between the Agency and healthcare professionals that particularly focused on the interaction with their professional organisations.

The Agency is also developing collaboration with academia with a framework expected to be endorsed by end of 2016. More information on this framework will be provided in section 3.2.2.

The topic group on academia, learned societies and healthcare professionals' organisations consists of HCPWP members and relevant EMA staff. The key objectives include:

- Map organisations' current practices/ initiatives intended to promote involvement in regulatory activities and raise awareness of that involvement amongst their members
- Brainstorm around group Vs individual approaches in relation to interaction with EMA
- Support development of the EMA framework of collaboration with academia
- Reflect on the need to review the EMA framework of interaction with healthcare professionals
- Prepare recommendations to EMA and to healthcare professional organisations intended to raise awareness of how the EU Medicines Regulatory Network functions (by Q4/2016)

The topic group kicked off in June 2015. The group recommended that the 2011 Framework of interaction with healthcare professionals should be updated to include a more relevant role of healthcare professionals in drug development and monitoring and align it with the expected framework of collaboration with academia. Furthermore, it provided input on which initiatives could enhance relationships with academia.

### **3.2.2.3. Workshops, meetings and consultations**

This section includes additional interactions with healthcare professionals, which were not covered in section 1.6. . A full overview of EMA workshops, conferences, ad hoc meetings and consultations involving healthcare professionals' organisations can be found in table 13.

**Table 13: EMA activities involving healthcare professionals' organisations**

<b>Activities involving healthcare professionals' organisations</b>	<b>Number of representatives</b>
Ad-hoc observers/experts attending HCPWP meetings	8
Observer at PCWP meetings	2
Scientific Committees/Working Parties consultations with HCPOs	8
Comments to EMA draft guidelines, concept papers and reflection papers	2
EMA input on regulatory consistence of ILAE EAN recommendations for use of valproate in female patients	1
Regulatory restrictions concerning the use of bromocriptine (teleconference) with Heart Failure Association (HFA) of the European Society of Cardiology (ESC)	2
Clinical trial Regulation programme subgroup 5 – public view (teleconference)	2
PCWP/HCPWP social media topic group survey	27
Excipients in the label and package leaflet of medicinal products for human use (Sodium Lauryl sulfate; Fragrances)	1
Feedback on risk management plan (RMP) summaries	26
Cross-Committee Task Force on patients registries (teleconferences and meeting)	8
Draft proposal for an addendum, on transparency, to the "Functional specifications for the EU portal and EU database to be audited"	1
EU clinical trials portal and Union database stakeholders' meetings	6
EMA Perception Survey	43
Coordination meetings with HCP representatives of Scientific Committees	10
Science, Medicines, Health: Patients at the heart of future innovation conference	13

Activities involving healthcare professionals' organisations	Number of representatives
Follow-up stakeholder meeting on the implementation of EMA policy on publication of clinical data	2
Implement. policy on access to clinical data - Meeting with concerned stakeholders on anonymisation and CCI	1
Workshop on haemophilia registries	4
Anticoagulants workshop	2
EMA workshop on the development of new medicinal products for the treatment of ulcerative colitis and Crohn's disease	1
Workshop on biosimilars	17
Workshop on chordoma as a model for very rare cancers	1
Bacteriophage therapy workshop	2
Workshop to clarify concept/demonstrating of significant benefit of orphan medicines	4
Webinar: Implementation of EMA policy on publication of clinical data	1
9th Stakeholder Platform meeting on the implementation of the pharmacovigilance legislation	3
EMA workshop on shortages	3
TOPRA meeting	1
EMA 20th anniversary event - The view from the sharp end: what patients and healthcare professionals can do for us	1
IMI ADVANCE project	2
Joint EMA-EBE (European Biopharmaceutical Enterprises) seminar	1
Consultation on EMA reflection paper on a proposal to enhance early dialogue to facilitate accelerated assessment of priority medicines (PRIME)	1
<b>TOTAL</b>	<b>207</b>

### Workshop on the therapeutic use of bacteriophages

In light of the growing challenge of antimicrobial resistance, the EMA is taking steps to facilitate the development of alternatives to currently available antibiotics. A [workshop on the therapeutic use of bacteriophages](#) was held to explore opportunities for new anti-bacterial treatment options and to facilitate the development of new tools for difficult-to-treat infections; including those due to multi-resistant bacteria. Bacteriophage are viruses that parasitise a bacterium by infecting it and reproducing inside it.

This workshop brought together experts and stakeholders from the academic, industrial and regulatory sectors to discuss different aspects of developing bacteriophage therapies for treatment of bacterial infections. The aim was to proactively discuss major issues including regulatory aspects and reflect on potential ways forward for this alternative therapy. Bacteriophage therapy is used in some parts of Europe, but is currently not authorised as a medicinal product.

The workshop focused on providing an overview and quality requirements for phage production and clinical development for phage therapy.

Representatives from the Standing Committee of European Doctors (CPME) and of the European Wound Management Association (EWMA) attended and contributed to the workshop.

## Bilateral interactions

There were also cases where healthcare professional organisations contacted the Agency to ask for input or address their concerns. These resulted in bilateral interactions between specialists within EMA and the organisations' representatives.

### *Valproate medicines*

As a follow up to the discussions regarding the review of valproate medicines in 2014, the Agency was contacted by representatives of a joint Task Force of ILAE-Commission on European Affairs and European Academy of Neurology (EAN) to discuss their [manuscript](#) for the use of valproate in female patients in the light of EMA's referral procedure outcome and ensure regulatory consistency. The review of valproate medicines was carried out following the publication of new data on the risks of malformations and developmental problems in babies exposed to valproate in the womb. As an outcome of the review, doctors in the EU are now advised not to prescribe valproate for epilepsy or bipolar disorder in pregnant women, in women who can become pregnant or in girls unless other treatments are ineffective or not tolerated. Those for whom valproate is the only option for epilepsy or bipolar disorder should be advised on the use of effective contraception and treatment should be started and supervised by a doctor experienced in treating these conditions.

### *Oral Bromocriptine*

As an outcome of a referral procedure in 2014, the Coordination Group for Mutual Recognition and Decentralised Procedures – Human (CMDh) recommended to only prescribe bromocriptine-containing medicines by mouth to prevent or suppress breast milk production when there are compelling medical reasons. The reasons for not routinely prescribe bromocriptine are the severe side effects, including women with various disorders that increase blood pressure or who have or have had heart disease or severe psychiatric disorders. Following this outcome, the Heart Failure Association (HFA) of the European Society of Cardiology (ESC) expressed concerns on precluding the possibility to treat women with peripartum cardiomyopathy (PPCM), a complication of pregnancy that could be fatal, as they strongly believe that a low dose of oral bromocriptine would be justified for these patients. The EMA organised a teleconference with HFA representatives in March 2015 to clarify their concerns on the restricted use of oral bromocriptine for prevention and suppression of lactation and discuss its implications for the use of bromocriptine in PPCM.

## Participation in written consultations addressing specific issues related with real 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 matters (not product-specific consultations). 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 14 lists the consultations carried out in 2015.

**Table 14: Committee/Working party consultations with healthcare professional organisations**

Committee/ WP	Subject	Contribution of healthcare professionals
PRAC	Input on a set of questions related with the use of codeine containing medicinal products for the treatment of cough and/or cold in paediatric patients, in the context of a referral procedure (Art. 31)	The PRAC obtained additional information from European healthcare professionals' organisations (HCPO) on the paediatric population which could benefit from the use of codeine in the symptomatic treatment of cough and/or cold.

		Overall, HCPOs were of opinion that there was no specific paediatric age group or condition that could benefit from the use of codeine as an antitussive. However, the use of codeine in cases of persistent irritating cough that is resistant to other antitussives was suggested. It was also stated that there would be no detrimental impact if codeine was to be restricted in the paediatric population. Clinical experience did not demonstrate any known risks with alternative antitussives but the range of medications used (including unconventional and herbal medications) is very wide and these medications may have associated concerns.
PDCO	Consultation on the publication of the PDCO public summaries of the evaluation of PIPs	The PDCO gathered information from HCPOs on their awareness of these summaries.
PRAC	Draft guidance & communication on risk minimisation strategies for medication errors with high strength and fixed combination insulins	The PRAC obtained input from HCPOs on the proposed guidance and key communication messages of the strategy and how organisations can join efforts in awareness and understanding of proposed measures

### 3.2.3. 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.

#### 3.2.3.1. Healthcare professional involvement in scientific meetings

As described in Section 2.2.3.1.2. 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 21 individual experts to participate in SAG/ad –hoc expert group meetings and bring additional expertise on clinical practice in specific domains during 2015. This expertise was provided on a variety of therapeutic areas and medical fields, including alpha1-proteinase inhibitor deficiency, anaphylaxis (severe allergic reactions), malaria, attention deficit hyperactivity disorder (ADHD), multiple sclerosis, lipodystrophy, and urea cycle disorders. Experts also participated in SAGs and ad-hoc expert group meetings specifically convened in the context of safety referrals covering the review of adrenaline auto-injectors and human papillomavirus (HPV) vaccines.

#### 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;
- giving advice on geriatric aspects of the development, assessment or safety monitoring of medicines;
- 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 2015 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.

### 3.2.3.2. 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 2015.

**Table 15: Committee/Working party consultations in writing with healthcare professional (individual experts)**

Committee/ WP	Medicinal product	Contribution of healthcare professionals
PRAC	Good Practice Guide on Risk Minimisation and Prevention of Medication Errors - Addendum on Risk minimisation strategies for high strength and fixed combination insulin products	Input on the guide to prevent medication errors
EMA/QRD	Ophthalmic formulation	Input on expression of strength to avoid medication errors and on product information and pack design
EMA/QRD	Hypophosphatasia medicine	Optimal ways to express the strength to allow identification and differentiation between presentations to minimise the risks of medication errors at the level of prescribing, dispensing and/or administering
EMA/QRD	Anticancer medicine	Identify the best standard term to identify the pharmaceutical form in order to help ensuring the correct preparation of the product for its safe use
EMA/QRD	Heart failure medicine	How to minimise the risks of medication errors at the level of prescribing, dispensing and/or administering due to the suprabioavailability of one of the components of the fixed dose combination, compared to the approved mono-component, as a switch from one product to the other is possible, considering that both products have overlapping indications
EMA/QRD	Ophthalmology medicine	How to minimise the risks of medication errors at the level of administration of the product due to the expression of the strength, overfill and injection technique
EMA/QRD	Anticancer medicine	How to minimise the risks of medication errors with liposomal formulations on labelling and product information

### **3.2.3.3. 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 2015, 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 2015, a total of 13 experts nominated by healthcare professional organisations (HCPOs) with different specialities and clinical backgrounds were involved in the review of 25 safety communications, of which four related to medication errors, and 4 DHPCs. Most of the feedback received was positive with pertinent suggestions used to reinforce the clarity of the messages to be conveyed.

An example where healthcare professionals contributed to the shaping of the risk communication messages related to the referral of codeine containing medicines when used for cough and cold in children (aged below 18 years). This referral was initiated in 2014 upon request of Germany based on a previous review of these medicines when used for pain relief in children, which was triggered by concerns over the risk of morphine toxicity. During the evaluation healthcare professionals were consulted and agreed that codeine is no longer considered an essential therapeutic option for cough in children. In 2015 it was decided that codeine should not be used in the treatment of cough and cold in children because of the risk of serious side effects with these medicines including the risk of breathing

problems. Healthcare professionals reviewed both safety communications after PRAC and [CMDh recommendations](#) on readability and understand ability for healthcare professionals.

In addition, eight healthcare professionals were involved in the review of 4 DHPCs. These concerned an anti-cancer medicine; a radiopharmaceutical (a medicine containing a radioactive substance) intended to treat cancer in the prostate; a specific combination of hepatitis C medicine and an antiarrhythmic medicine and a medicine to treat non-small-cell lung cancer.

### 3.2.1. Communication on shortages

As previously described, the online [public catalogue](#) on shortages is available and patients, consumers and healthcare professionals review the information prior to publication. In addition, once the Agency is informed of a shortage of a medicine, it prepares a draft 'Direct healthcare professional communication' (DHPC) that is also reviewed by healthcare professionals.

In 2015, the shortage communications on [Xofigo](#) (radium-223 dichloride), used to treat men with cancer of the prostate, and [Tygacil](#) (tigecycline), which is used to treat adults with complicated infections of the skin and soft tissue, were reviewed by healthcare professionals. Furthermore was the 'Direct healthcare professional communication' (DHPC) of Xofigo reviewed by a healthcare professional.

### 3.2.2. Interaction with Academia

The Agency has had a long standing interaction and collaboration with academia, which has not been fully visible outside or formalised within the Agency. In view of the growing complexity with which new medicines are being developed, evaluated and monitored it has become indispensable for regulators to strengthen the partnership with academia and foster a proactive process to support innovation and channel it into the continuous evolution of the regulatory environment. This priority is fully reflected in the EU Medicines Agencies [Network strategy](#) to 2020 that highlights the importance of developing a sound collaboration between the EU Regulatory Network and the academic world. Therefore, the EMA will develop a framework of interaction with academia in the same vein as has already been done with other stakeholders.

As part of the process, an internal survey was conducted in March 2015 to collect information on the current interactions between the Agency and academia; furthermore, a brainstorming on the key components of the future framework was organised with healthcare professionals and learned societies in the context of the HCPWP meeting in June 2015. Informal exchanges (via teleconference) and face-to-face meetings (August 2015-December 2015) have also been organised with representatives of several European research infrastructures operating in the biomedical field and with other academic organisations to open a dialogue focused on identifying the pillars on which the framework should be conceived and built upon. In parallel, a consultation process has been initiated with the academic world at large by elaborating and finalising a questionnaire (December 2015) that will be launched at the beginning 2106. This consultation process has the following objectives:

- Explore opportunities for a greater collaboration in order to better support academia in generating new medicines that meet regulatory standards;
- Channel academia's advanced knowledge into the regulatory environment;
- Assess the degree of awareness among academics of the existing activities and incentives provided by regulators to support medicine development;
- Refine regulators' understanding of academia's needs and expectations and develop a methodology for collaboration.

The results of the survey will provide points of reflections and will input into the definition of the framework of collaboration. The aim is to have the framework ready for adoption by the EMA Management Board at the end of 2016.

### 3.3. EMA awareness-raising activities

In order to promote further awareness on how the Agency is involving healthcare professionals in its activities, the Agency participated in several specific meetings and conferences organised by healthcare professionals' organisations in 2015, as shown in table below.

**Table 16: EMA participation in external healthcare professionals' meetings**

Organiser/Event	
1	European Association of Hospital Pharmacists (EAHP) - 20 <sup>th</sup> Annual Congress: <i>"How can information about medicines shortages be improved?"</i>
2	European Forum for Primary Care (EFPC) – International conference
3	European Academy of Allergology and Clinical Immunology (EAACI)- High level stakeholders lunch
4	European Haematology Association (EHA) – 20 <sup>th</sup> Congress
5	The World Organization of National Colleges, Academies and Academic Associations of General Practitioners/ Family Physicians (Wonca) Europe – Conference 2015
6	European Specialists Nurses Organisations (ESNO) - Summit

#### 3.3.1. Reaching out to 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.

Following a first workshop with members of the EFPC during their 2015 conference in Amsterdam, further progress was achieved at the 20<sup>th</sup> WONCA Europe conference in Istanbul, where a meeting was organised between EFPC, the European Union of General Practitioners (GPs)/ Family physicians (UEMO), WONCA Europe and EMA.

All three organisations recognised the importance of bringing the general practitioners perspectives and experience into the EMA regulatory discussions and welcomed the EMA proposal to hold a dedicated workshop with GPs in 2016. Agreement on the topics to be discussed was also reached.

The aim of the workshop is to open up bidirectional avenues that can:

- help EMA to gain a better understanding of how medicines are being used in real clinical practice and the potential impact of specific regulatory actions on patient care
- support the creation of more awareness amongst GPs on how they can better inform regulatory discussions on benefit-risk evaluation of medicines and promote the alignment of regulatory decisions with the reality of clinical practice.

#### 3.3.1. Reaching out to specialised nurses

The EMA attended the European Specialist Nurses Organisations (ESNO) Summit and gave a presentation on how EMA is interacting with healthcare professionals. Examples of where the input of

specialist nurses could contribute to bringing real-life experience into the evaluation of medicines were illustrated.

The summit was an opportunity to gain a better understanding of the issues faced by specialist nurses in terms of recognition of specialisations and mobility of nurses in Europe as well as to learn about ESNO's initiative to set up a nursing medication reference group as a means to input further into EMA consultations.

### 3.4. Organisations involved in EMA activities in 2015

There were no changes in the list of EMA eligible organisations in 2015. The 29 healthcare professionals' organisations are shown in 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](#)'.

Table 17 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.).

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](#)'.

**Table 17: 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) <input type="checkbox"/>
3	European Academy of Neurology (EAN)
4	European AIDS Clinical Society (EACS) <input type="checkbox"/>
5	European Association for Clinical Pharmacology and Therapeutics (EACPT)
6	European Association of Hospital Pharmacists (EAHP) <input type="checkbox"/>
7	European Association for the Study of Diabetes (EASD)
8	European Association of Urology (EAU) <input type="checkbox"/>
9	European College of Neuropsychopharmacology (ECNP)
10	European Federation of Internal Medicine (EFIM) <input type="checkbox"/>
11	European Forum for Primary Care (EFPC)
12	European Haematology Association (EHA) <input type="checkbox"/>
13	European League Against Rheumatism (EULAR)
14	European Renal Best Practice (ERBP) <input type="checkbox"/>
15	European Society for Medical Oncology (ESMO)
16	European Specialist Nurses Organisations (ESNO) <input type="checkbox"/>
17	European Society of Cardiology (ESC)
18	European Society of Endocrinology (ESE) <input type="checkbox"/>
19	European Society of Oncology Pharmacy (ESOP)
20	European Stroke Organisation (ESO) <input type="checkbox"/>

Name of Organisation	
21	European Society of Radiology (ESR)
22	European Union of General Practitioners / Family physicians (UEMO)□
23	European Union Geriatric Medicine Society (EUGMS)
24	European Working Group on Gaucher Disease (EWGGD)□
25	Health Care Without Harm Europe (HCWH Europe)
26	International League Against Epilepsy (ILAE)□
27	Pharmaceutical Group of the European Union (PGEU)
28	Standing Committee of European Doctors (CPME)□
29	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 18: List of organisations consulted by EMA on specific areas**

Name of Organisation	
1	Centro Emofilia & Trombosi Angelo Bianchi Bonomi - International Society on Thrombosis and Haemostasis (ISTH)
2	European Association for the Study of the Liver (EASL)
3	European Haemophilia Safety Surveillance (EUHASS)
4	European Kidney Health Alliance (EKHA)
5	European Psychiatric Association (EPA)
6	European Respiratory Society (ERS)
7	European Society for Pediatric Gastroenterology, Hepatology and Nutrition (ESPGHAN)
8	European Society of Clinical Microbiology and Infectious Diseases (ESCMID)
9	European Society of Ophthalmology (SOE)
10	Rare cancers Europe
11	European Wound Management Association

### **3.5. Next steps**

In 2016, the Agency will continue to focus on the following areas:

- Revision of the framework of interaction with healthcare professionals;
- Follow up of HCPWP topic groups' deliverables;
- Development of a longer term planning linked to the EU Network Strategy to 2020.
- Setting up a framework of collaboration with Academia
- Implementation of public hearings

## 4. Conclusions

The quality of interactions and the extent of activities reported in this report are thanks to the commitment of the patients, consumers and healthcare professionals who engage with the Agency. While the EMA invests considerable time and effort into ensuring that the voice of these stakeholders is represented in all aspects of its work, it is a partnership that is rewarding and has been vital for continued success.

While these interactions are well established, there is still work to be done to ensure that the right expert is identified for scientific meetings, workshops and consultations and that the right level of support is provided throughout the interaction with proper follow up.

Added efforts to include young people, general practitioners, and academia in the discussions will be made and a framework for collaboration with the latter will be initiated. In this vein, the existing framework for interaction with healthcare professionals will be revised. The topic groups that began this year will be continued until recommendations are elaborated and the deliverables will be monitored. In addition, the EMA will continue to strive to:

- Maintain interest and levels of participation in meetings, surveys, working groups;
- Provide contextualised information on its activities to support meaningful engagement;
- Find the balance between real life experience of a condition, clinical practice and academic/research interests.

The implementation of public hearings in 2016 is an exciting tool in engagement with European Union citizens and to listen to their views and experience. The EMA is planning on holding public hearing in the fourth quarter of 2016.

In the longer term, planning linked to the EU Network Strategy to 2020 will be developed. All the above elements will need to be integrated in the full implementation of the EMA's transparency requirements for patients', consumers' and healthcare professional organisations as well as the renewal of the working party mandates and election of the co-chairs, to ensure the smooth continuity of the involvement of these stakeholders in EMA core activities.