



EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

Stakeholder Engagement report 2017

Patients, consumers, healthcare professionals,
academics and their organisations

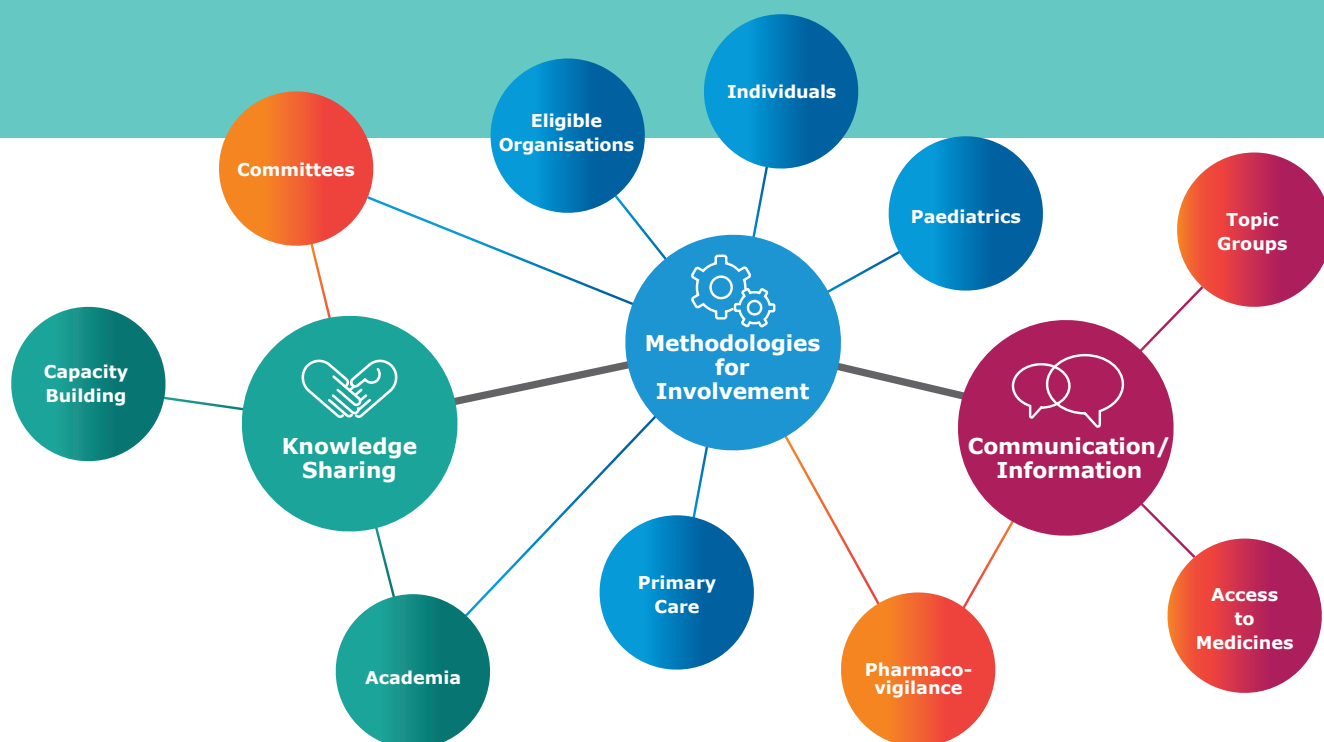


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Executive summary

As EMA prepares for its relocation and the UK's withdrawal from the EU, the Agency launched the first phase of its business continuity plan (BCP) in 2017. As a result work in some areas has been reprioritised, suspended or postponed to resource Brexit preparedness activities and safeguard core activities. This approach to BCP has enabled EMA to maintain an effective level of stakeholder engagement in 2017 and underlines our commitment to better public health. This report charts the progress made in several key areas.

A particular priority was the organisation of the first public hearing held on September 26th. The event brought together patients, carers, healthcare professionals and other stakeholders to discuss additional measures to minimise the risk of valproate-containing medicines. The input from the public has been instrumental in finalising the assessment of valproate and recommending new measures to protect women at risk.

Other public engagement highlights include:

- ▶ Critical input was also obtained from stakeholders through dedicated meetings during the assessments of retinoids and valproate. Patients and healthcare professionals provided their expert opinions and experience to assist the PRAC to make recommendations.
- ▶ Agreement to involve patients in benefit – risk discussions in CHMP oral explanations; the pilot has been concluded and a report has been published.
- ▶ The Agency's Management Board agreed on Principles on involving young people in Agency activities; the Agency is one of the first medicines regulatory authorities to formally advance in this area, providing a framework that facilitates the collection of input from this important group of patients.
- ▶ Further dialogue with different groups representing general practitioners, building on the 2016 workshop, with the aim of enhancing and improving their involvement in regulatory activities.
- ▶ More streamlined and efficient support for EMA committee members representing patients and healthcare professional is now being explored to assist them in fulfilling their role within the committees.
- ▶ Complementing the existing portfolio of frameworks, the management board of the Agency adopted the framework for collaboration with academia. The implementation phase has begun according to an action plan that will be updated and refined as needed.
- ▶ A total of 131 patients participated in scientific advice procedures, which also includes 18 joint advice procedures provided with health technology assessment (HTA) bodies.

To comply with the restrictions of the BCP, the activities of the Patients and Consumers Working Party (PCWP) and Healthcare Professional Working Party (HCPWP) have been better aligned. A joint work plan for 2018/2019 was drafted in collaboration with members from each working party. Priorities within this work plan have been reorganised in order to cope with the challenges ahead; however it still remains in accordance with the work programme to 2020 of EMA and the common strategy of the European regulatory network.

The working parties focused their efforts on two main topics: personalised medicine and antimicrobial resistance, and two dedicated workshops were organised. Personalised medicine is a complex topic; policy developments and clinical practice as well as the role of all players were discussed. The second workshop on antimicrobial resistance aimed to engage in an exchange of ideas on how to join efforts to improve education and communication on antimicrobial resistance. Patient, consumer and healthcare professional organisations have extensive outreach through their members and can cascade this information to them as well as bring their perspectives to the conversation. It is thus essential that these and similar conversations are brought to the working parties' platform for discussion.

There were a total of 925 activities involving patients and 445 activities involving healthcare professionals during 2017. Despite the limitations imposed by Brexit preparedness and BCP, this has meant the Agency has continued to experience some benefit from the input of patients and healthcare professionals into its activities. As success is not only counted in terms of total numbers, the report focuses mainly on the added value of their input.

To streamline and simplify reporting, the format of this year's annual report has been revised to present information in a briefer and more contextualised fashion. The words of members our eligible organisations can be found throughout the report, complementing the activities described by the Agency.

Future steps

Next year will bring its own challenges as preparations to relocate the Agency to Amsterdam are stepped up and BCP enters the next phase. Activities will be reduced in 2018 and more so as we approach 2019. However, the Agency remains committed to continuing to provide the necessary support for patients, consumers, healthcare professionals and academics to participate in relevant EMA activities as far as possible.

The process of renewal of eligibility for organisations working with EMA will be streamlined and simplified to make it easier for both EMA and organisations.

Despite a reduction in the number of interactions that are foreseen in 2018, we aim to continue our valuable work with the patient, consumer and healthcare professional working parties, and implement their work plans. However, the number of meetings will be reduced and topic groups will only operate where needed.

EMA will continue to explore previously tested methodologies such as the elicitation of patient preference studies to support benefit-risk evaluation. We will also update the action plan of the framework of interactions with patients and consumers to align it with the current environment and future challenges. Implementation of EMA's Action Plan to improve Product Information will also be delayed due to Brexit and BCP; in 2018 and 2019, only the proposed actions on electronic product information will be progressed.

Specifically for healthcare professionals, we will continue to facilitate their early engagement in risk minimisation activities and initiate discussions on how to further promote inclusion of general practitioners in EMA activities.

With all stakeholders in mind, the experience of the first public hearing will contribute to a published report on the lessons learnt and these lessons will be implemented in future public hearings; a future hearing has already been planned for June 2018. Beyond this, EMA will no longer be in a position to organise further public hearings until EMA reverts to being fully operational.

This report was presented to the Management Board during its meeting on 6 June, 2018.

2017 highlights

Personalised medicine

Workshop describing some policy developments and how clinical practice and public participation can support personalised medicine in EU regulatory activities



Antimicrobial resistance

Information session intended to raise awareness of the work of EU institutions, member states and WHO in the fight against AMR



Involvement of young people

Adoption of principles document enabling the inclusion of young people in discussions at EMA



Patients in benefit-risk discussions at CHMP

Publication of report on CHMP pilot to include patients in benefit-risk discussions at CHMP meetings



Academics and researchers

Framework of collaboration adopted by EMA Management Board and implementation of action plan initiated



EMA annual training day

Training day extended to include healthcare professionals and young people for the first time



Update of Product information - Commission report

Recommendations identified to improve documents and meet needs of stakeholders - EMA creates an action plan



Public hearing

EMA holds its first public hearing on a safety referral



1. Highlights of areas of common interest and collaboration in 2017

1.1 Introduction

In this first chapter, we highlight and describe areas of common interest and collaboration for patients, healthcare professionals and academics. The overarching themes, as shown in Figure 1, include i) a continual sharing of knowledge and building of capacities, ii) improving and expanding methodologies for engagement and iii) targeted communication and information, relevant to and adapted for the specific stakeholder group concerned. These are by no means all of the existing connections but serve to provide an overview of the structure of chapter one.

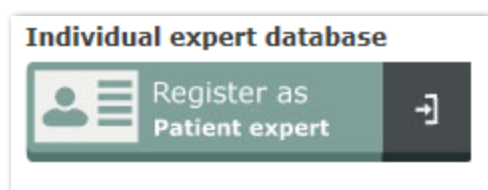
More detail is provided in subsequent chapters that are dedicated to each group; patients and consumers (Chapter 2), healthcare professionals (Chapter 3) and academia (Chapter 4).

Figure 1. Mapping EMA activities and stakeholder engagement



1.2 Engage, Involve, Participate: expanding methodologies

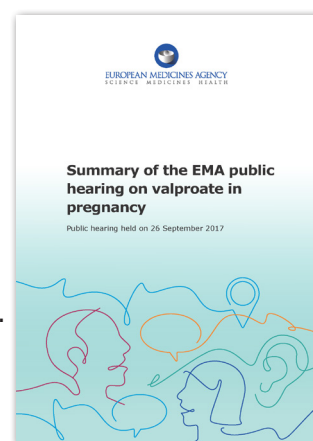
Involvement of stakeholders such as patients, consumers and healthcare professionals, in EMA activities is not a 'one size fits all' methodology; however, the overarching principles of the stakeholders relations management framework of [inform, consult, consult/involve and cooperate/participate](#) apply to all. To achieve this, specific frameworks describing how to interact and collaborate with patients and consumers, healthcare professionals, industry¹ and most recently academics exist. Through the frameworks, we engage with an extensive group of organisations interested in working with EMA and have also recently put in place a database for individual patients wishing to collaborate with EMA to register. Information on individuals in EMA activities is provided in the following chapters. These **organisations** are kept informed of and consulted on relevant guidelines and concept papers (2.4.2 and 3.4.2) and are also invited to participate in specific workshops.



Organisations have also contributed to medicine related **stakeholder meetings** and two such meetings regarding the safety of specific groups of medicines were held in 2017. The first, held in March, saw representatives from patient and healthcare professional organisations invited to a meeting along with members of the Pharmacovigilance and Risk Assessment Committee (PRAC) and EMA staff regarding a referral procedure for **retinoid-containing medicines**. The expertise of these stakeholders was requested on issues from pregnancy prevention plans (tools and measures) to dispensing medicines and compliance of use. Input and perspectives of patients and healthcare professionals were taken into account in the subsequent recommendations issued by the PRAC.

The second stakeholder meeting, held in October, was related to a referral procedure for **valproate-containing medicines**. Healthcare professional and patient organisations representing epilepsy, bipolar disorder and migraine as well as organisations representing the patients, families and carers who have been affected by valproate were invited to participate and provide their input and experience on a series of questions posed by the PRAC. Questions around the information provided by healthcare professionals to patients on the risk of use of these medicines during pregnancy, to the best way to present this information to measures and actions to increase the awareness of the risks were discussed.

The content of the meeting was very much informed by the input received during the **public hearing** on valproate-containing medicines, which took place on September 26 [see following page]. This was the first public hearing held at the EMA and had been foreseen in the new pharmacovigilance legislation. In the case of valproate-containing medicines, patients and healthcare professionals representing the three treatment areas (mentioned above) for the medicines were invited to register to attend and to present their perspectives on specific questions aimed to assist the committee in reaching conclusions on minimising associated risks. There were a total of 65 attendees that included 28 patient representatives, 19 healthcare professionals and academics, 11 from the pharmaceutical industry and seven representing media. A summary [report](#), copies of all [written interventions](#) and a [recording](#) of the entire hearing are available.



¹ A report on EMA's interaction with industry stakeholders is prepared annually and published on the [website](#).



Public hearing

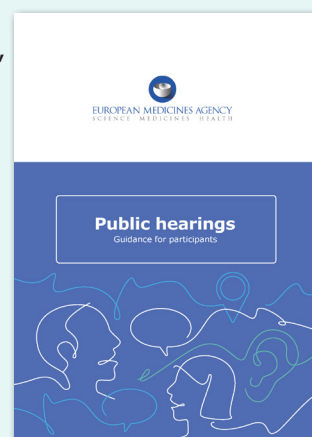
The first public hearing took place on September 26th 2017.

Public hearings are an additional engagement methodology and give a voice to EU citizens in the evaluation of the safety of medicines and empower them to express their views on issues related to the safety of certain medicines and the management of risks.

A public hearing is a way for EMA's safety committee, PRAC, to gain input and insights from the public and cover a specific concern or risk with a medicine or a group of medicines.

By working directly with people affected by taking a medicine or those who prescribe, treat and advise patients, EMA can increase its understanding of how it is used and make sure that EMA's actions to manage risks are appropriate and practical.

Public hearings are broadcast live and recorded enabling the general public to learn how EMA works and particularly how EMA aims to improve a medicine's benefits by minimising risks. Contribution from the public at hearings will inform PRAC's decision making. PRAC assessment reports will show how information from the hearings contributed to the overall evaluation of the medicine under consideration.



All European patient and healthcare professional organisations are welcome to register as an interested party and are kept informed of EMA activities. EMA closely interacts with organisations that fulfill the EMA criteria for eligibility. These eligible organisations are the first port of call when EMA is looking for input in relation to a particular area of interest, either as an organisation or by proposing individual patients or healthcare professionals. The logos of the eligible organisations are displayed on the EMA webpage along with links to their individual webpages. After the revision and implementation of the eligibility criteria for [patient](#), [consumer](#) and [healthcare professional](#) organisations last year, 2017 presented an opportunity to explore a simplification of the annual re-assessment of eligibility, which will be implemented in 2018.

Other key platforms for exchange of information are the working parties (Patients' and Consumers' Working Party and Healthcare Professionals' Working Party) (Sections 2.4.1 and 3.4.1) that are composed of eligible organisations. Their work plans are aligned with the [EU medicines agencies network strategy to 2020](#) and the [EMA multi-annual work plan](#). To reflect the common interests of the two working parties, a specific working group composed of representatives of patients, consumers and healthcare professional organisations has drafted a **joint work plan for 2018/19** in conjunction with EMA².

Many of the PCWP and HCPWP **topic groups** initiated in 2015 have now concluded their tasks and a [report](#) of their work has been published.

Highlights from this year include the re-launch of the Social Media topic group as the broader Digital Media and Health topic group. One of the objectives of the Involvement of young people in EMA activities topic group was achieved when the [Principles for the involvement of young patients/consumers within EMA activities](#) was adopted. The purpose of this document was to help young people in their future involvement with EMA and with this in mind, four young people were invited to participate in the **EMA annual training day** for the first time in 2017.

“

Meetings of EMA working parties, evolving topic groups and medicine-related updates provide excellent fora for education, for developing interactions with European healthcare professional and patient organisations and thus contribute to raising standards for safety of medicines for the European and wider public, a key goal for the European Association for Clinical Pharmacology and Therapeutics.

” **Donald Singer (EACPT)**

² [2018-2019 Work plan for the Patients' and Consumers' Working Party \(PCWP\) and the Healthcare Professionals' Working Party \(HCPWP\)](#)

Responding to queries from stakeholders

2017 saw an increase in queries to EMA with 838 from patients/consumers, 283 from healthcare professionals and 253 from academia/research institutes. Most of the questions were related to the availability of medicines and adverse effects. Approximately 20% of the queries were received from non-EU countries.

1.3 Better medicines for young people

Several activities in the field of paediatrics took place in 2017 that support the Paediatric Regulation, which aims to address gaps in knowledge on how medicine should best be used by children and to reduce off-label use and increase the number of medicines specifically developed and tested for children.

As with previous years, workshops addressing paediatric issues were organised and two workshops are described. A meeting of the joint EMA and ACCELERATE Paediatric Strategy Forum was held on anaplastic lymphoma kinase (ALK) inhibition in paediatric malignancies. The need to accelerate development of new medicines for children and adolescents with cancer was discussed. Future Forums are planned for other relevant oncologic paediatric diseases and targets with a high unmet medical need in order to support the introduction of innovative treatments into the standard care of children with very rare cancers. A [full report](#) of the Forum was published.

A joint workshop addressing unmet needs of children with [pulmonary arterial hypertension \(PAH\)](#) was held in June 2017. This was a multi-agency workshop organised by EMA/FDA/Health Canada. A wide range of stakeholders including patients, healthcare professionals and academics took part in this two day event and presented their various perspectives. An overview of PAH and its current management in children was given and the requirements for the development of medicines in PAH and to address the need of the current paediatric clinical practice in a timely manner were discussed. Further sessions were held to identify the need and requirements for PAH studies in children and to identify appropriate endpoints for clinical trials. Patient/parents views on clinical trials challenges were discussed as part of a session on future perspectives in this area. Copies of all presentations and a video recording of the workshop are [available](#).

Another important workshop was on the [extrapolation of efficacy and safety](#) in medicine development across age groups, which was based on a [reflection paper](#) for extrapolation of data from adults to children that could serve as a basis for regulatory decision-making. The primary rationale for extrapolation is to avoid unnecessary studies in the target population for various reasons.

The European network of paediatric research at EMA (EnprEMA) held their [9th annual multistakeholder workshop](#) where patients/parents, regulators and medicines developers came together. A [report](#) containing a full summary of this meeting has been published, however importantly one of the working groups is on how to engage and involve young people in clinical research. The newly formed European Young Persons Advisory Group (eYPAGnet) became a member of EnprEMA and good practises and training materials will be developed consistent with the **Principles** document also adopted by EMA.

A [10-year Report to the European Commission](#) on the experience acquired with the paediatric regulation was prepared by the EMA in conjunction with the PDCO. In October the European Commission published a [report on progress made in children's medicines since the paediatric regulation](#) came into force. Based on the findings in the report, the Commission, EMA and its Paediatric Committee will develop an action plan to improve the implementation of the Regulation.

These initiatives are essential and they complement the efforts being made to ensure that medicines for children are safe and tested in the right population, EMA is also making efforts to ensure that their voices are heard during the development of medicines to treat their conditions. The Principles document opens doors of engagement to young people affected by various conditions who can share their experience of the disease and its treatments, directly in EMA medicines' evaluation. Appropriate support and training materials will be developed to help them with their participation.

1.4 Strengthening collaboration with academia

An important stakeholder in the field of medicines development and research is academia. In order to support and strengthen their involvement in the regulatory sphere, a framework of collaboration with

academia was adopted this year. The framework is accompanied by an action plan that will allow the initial phase of its implementation. More details can be found in Chapter 4.

1.5 Knowledge sharing and capacity building

There is a need for regulatory processes to be understood by all stakeholders, 2017 saw the largest number of stakeholders being invited to the Agency for its **annual training day**. As the 10th training day to be held, the decision was made to extend the invitation to include healthcare professionals, young people (as mentioned above) and academics.

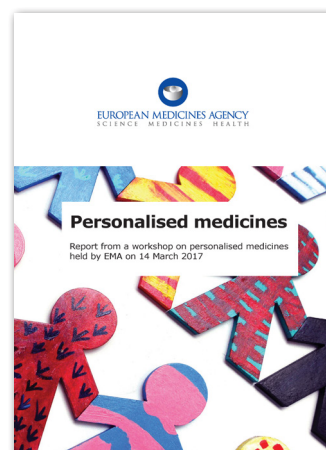
Over the years the training has evolved both in terms of content and delivery has been changed into a more interactive training with breakout sessions demonstrating real examples of where patients and healthcare professionals can be involved in EMA activities. The input gained from young people and healthcare professionals from this year's session will be used to specifically adapt the training materials and format according to their respective needs.

The breakout sessions are selected based on areas where both patient and healthcare professional involvement is sought by EMA; **scientific advice, scientific advisory groups and review of documents**. Given the nature of medicines development it is not possible to predict the therapeutic areas in which patient and healthcare professional input will be required. For this reason, the annual training day is used to train as diverse as possible a group of people possible with the objective of introducing them to the work of the Agency and demonstrating in which activities they can be involved and the added value of their expertise and experience.

In keeping with this theme of sharing knowledge and building capacities, EMA organised two key meetings relevant to advancing the health of citizens in Europe and beyond.

A first [workshop](#) on **personalised medicines** was organised following requests from the PCWP and HCPWP. The objective was to explain terminology, concepts and challenges relating to this area. Two presentations had previously been made to the working parties in 2016 setting the scene regarding the far-reaching implications of this area that encompassed the work of several of the topic groups as well as previous workshops such as big data, advanced therapies and patient registries.

The European Commission placed the importance on this subject by extensive funding of research in the area as well as initiating the International Consortium for Personalised Medicine (ICPerMed).

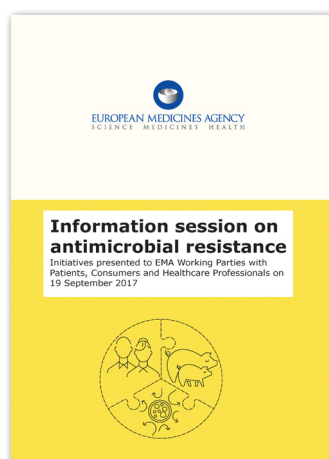


“EMA is piloting projects in personalised medicine which overlaps to a great extent with the interest of the European Hematology Association. We thank EMA for this initiative and hope that access to personalized treatment can be gained all over Europe.

” **Ulrich Jäger (EHA)**

As this is a topic of broad interest, many stakeholder groups took part in the workshop and speakers included representatives from the European Commission, the US Food and Drug Agency and the European Personalised Medicines initiatives. A representative from the European Dravet Syndrome Federation presented the patient perspective and a speaker from the European Haematology Association provided insight into the views of healthcare professionals on this subject. The full report and a video recording of the workshop have been [published](#).

To address a global concern, an information session on **antimicrobial resistance (AMR)** was organised. The emergence and increase of microbes resistant to antimicrobial treatments is a public and animal health issue. This [meeting](#) was organised in close collaboration with the European Centre for Disease Prevention and Control (ECDC) to raise awareness of the work of European Union institutions, Member States and WHO in the fight against AMR. A representative of the World Health Organization (WHO) described the global action plan to tackle AMR and perspectives were provided from both the human and veterinary sides of the issue.



The meeting also facilitated an exchange of ideas with patient, consumer and healthcare professional organisations on how to work together to improve communication, education and training in the fight against AMR. This included presentations from GPs (family physicians), community pharmacists, nurses as well as EPHA and ESCMID. Copies of all presentations and a video recording of the meeting are [available](#).

“ Antimicrobial resistance is a contemporary issue which should concern everyone. This became evident at the top-class session which was very well organized. Among scientists and health care professionals the patient representatives felt highly acknowledged for their expert knowledge and their input in the discussions.

” **Karl Philipp Drewitz (EMENA)**

To complement patients and healthcare professionals involvement in EMA meetings EMA staff also participate in **stakeholder-organised meetings**. This activity is important for increasing outreach and awareness of EMA's work, as well as for building and maintaining trust in the regulatory processes.



1.6 Access to medicines and information

While the mandate of EMA is to ensure that the medicines on the market are safe and effective, regulators have tried to implement more flexibility to meet the needs and expectations of the patients and healthcare professionals with the aim of having promising medicines available at the earliest appropriate opportunity.

Several EMA initiatives have been put in place to encourage medicines developers to address unmet medical needs for patients without treatment options and these have been discussed during PCWP/HCPWP joint meetings in 2017.

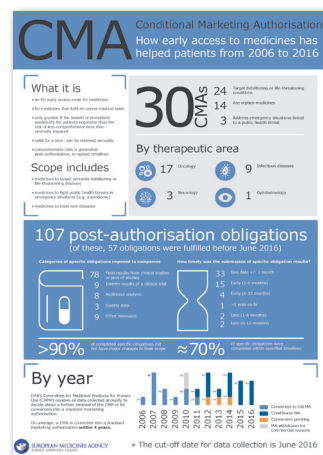
One example is the **PRIME** scheme, launched in 2016, and a [one year assessment](#) of the programme identified success factors for applicants as well as areas for improvement. Using existing regulatory tools, PRIME provides scientific advice and accelerated assessment to optimise development plans for new medicines that bring a major therapeutic advantage to patients. Small to medium-sized enterprises and academic developers are also supported with this scheme as EMA puts more attention on its support and engagement with [academia](#) (Chapter 4).



A [first anniversary meeting](#) aimed to review experience gained with PRIME, receive feedback from users, provide information on application of eligibility and support. A panel discussion of stakeholders took place and patients emphasised the need to incorporate the patient perspective, not only on scientific advice procedures, but also through involvement in eligibility procedures. A full report and video recording of the meeting is [available](#).

Another regulatory tool to support early access is the [conditional marketing authorisation](#), which allows for less comprehensive data at an early stage with a view to additional collection of data confirming the positive benefit-risk balance.

Conditional marketing authorisations are a result of a legislative change and are intended for medicines that address an unmet medical need, for medicines where the benefit of their immediate availability for patients outweighs the risk of less comprehensive data than normally required.



A [report of the 10 years](#) of experience with conditional marketing authorisation was published that provides details of the 30 conditional marketing authorisations that have been granted.

The next step before the medicine reaches the patient involves pricing and reimbursement decisions. These are adopted by healthcare **payers** at the national and regional level. These decisions are often prepared and supported by (cost-) effectiveness and relative effectiveness assessments of the medicine, an activity performed by **HTA bodies**.

The Agency recognises that a close interaction between regulators, [HTA bodies](#) and other relevant bodies is critical to enable patients' access to important new medicines and hence for the benefit of public health. EMA has been working closely with HTA bodies since 2012 and with the European Network for Health Technology Assessment (**EUnetHTA**) since 2010. A [joint work plan](#) for EMA and EUnetHTA was published that outlines key areas of collaboration. One objective of the [joint action 3 of the EUnetHTA](#) is to explore where and how patients, consumers and healthcare professionals can be involved in their work and one way of addressing this is to learn from the EMA experience with the PCWP and HCPWP.

HTA bodies have been consulted in parallel scientific advice procedures (since 2012); patients and to some extent healthcare professionals are routinely invited to participate in these meetings to provide their unique perspective on living with a condition and its treatments as well as their considerations on the development plans (Sections 2.5.1 and 3.5.1).

To complement this collaboration with HTA bodies, a meeting between [EMA and EU healthcare payers](#) was held for the first time in 2017. The objective was to explore synergies and foster mutual understanding and cooperation to help improve timely and affordable access of patients to new medicines.

Unavailability and shortages of medicines are also major hindrances to patient access and this has been recognised as a priority topic in the [EU Medicines Agencies joint strategy to 2020](#). The European medicine regulatory network is increasingly confronted with supply challenges and shortages caused by manufacturing non-compliance, falsified or stolen medicines or a number of other factors. A [reflection paper](#) and [action plan](#) addressing manufacturing issues is being revised and the PCWP and HCPWP will contribute to its implementation by promoting best practices on communication of shortages.

Working closely with partners and stakeholders is key to ensuring the availability of new and well-established use medicines. In this vein, a [joint EMA/HMA task force](#) has been created to provide strategic support, advice for coordination and concerted approach to the Network on the availability of the medicinal products authorised in the EU. In addition, the Slovakian and Bulgarian European presidencies have both put the spotlight on this issue of availability.

The European Commission [STAMP](#) Expert Group on Safe and Timely Access to Medicine for Patients was established in 2015 and provides advice and expertise to Commission services on how to improve implementation of EU Pharmaceutical legislation and speed up access to innovative and affordable medicines. EMA's initiatives such as the PRIME scheme, adaptive pathways, experience with conditional marketing authorisations, compassionate use at EU level and registries have actively

contributed to discussions at the EU level. These topics have been incorporated into the 2018/2019 work plans for the working parties and will be further discussed in the next two years.

Following a public consultation, the European Commission has published an [assessment report](#) on current shortcomings in the summary of product characteristics and the package leaflet and how they could be improved in order to better meet the needs of patients and healthcare professionals. The Summary of Product Characteristics (SmPC) and the Package Leaflet (PL) are an integral part of a medicines authorisation; in the EU all medicines must have accessible information on their safe and appropriate use.

The report makes a number of recommendations on how to improve and better meet the needs of patients and healthcare professionals. A presentation was made to the PCWP and the HCPWP during the June joint meeting and comments and suggestions were gathered in preparation for a future workshop which will focus on electronic product information (due to take place in 2018).

[Biosimilar medicines](#) are a type of biological medicine that offer advantages to EU healthcare systems, as treatment alternatives that could improve patients' access to biological medicines with proven pharmaceutical quality. More than 10 years of clinical experience have shown that biosimilars are safe and effective. An [information guide for healthcare professionals](#) was developed in conjunction with a dedicated biosimilar topic group of the HCPWP. As a consequence of this guide, the European Specialist Nurses Organisation (ESNO) presented during **Member's Voice** of the joint working party meetings that they will prepare a follow up guideline for nurses to acquire an understanding of biological medicines including biosimilars. Patient and healthcare professional organisations also contributed to the annual meeting of the CHMP biosimilar medicinal products working party with interested parties, particularly focusing on how to continue to improve the level of awareness and understanding about biosimilars among key actors.

1.7 Data and evidence

The increase in technological developments and the quantity of information that is currently available will have an impact on development and regulation of medicines. Guido Rasi (EMA Executive Director) has said on many occasions that the development of medicines will become more complex and used three words to describe what he sees as key: 'complexity, opportunity and patients'. One aspect of what he was referring to was the technology that already is, and will become available, to diagnose and identify patients and also that will provide more opportunities for future developments.

To capture more broadly the possibilities, the PCWP/HCPWP Social Media topic group was transformed into the Digital Media and Health topic group and expanded its focus to i) mHealth, ii) social media and iii) real world evidence. This topic group comprises patients and healthcare professionals and one of its objectives is to identify points of concern on the three focus areas in relation to the evaluation and monitoring of medicines and to discuss how best to address these points. The first day of the first joint working party meeting in 2018 will be dedicated to this topic.

Healthcare professionals, in particular those working in primary care, have contact with a vast majority of the population and therefore have a leading role in the generation of real world evidence. EMA already works with organisations that bring together the views of different primary care practitioners but additional efforts are being pursued in conjunction with three European groups representing general practitioners to further collaborate on how to capture their input in personalised treatments, innovative medicines and risk management plans.

Another source of real world evidence is the use of registries and in 2017, two workshops were held around specific diseases; [cystic fibrosis](#) and [multiple sclerosis](#). The EMA Initiative for Patient Registries aims to optimise and facilitate the use of patient registries for benefit-risk evaluations and invites patients, HTA and reimbursement bodies as well as national competent authorities to convene to discuss these recommendations and action. Reports are available for both workshops.



On top of numerous helpful learnings and interesting opportunities for own contributions within the Patient and Consumer Working Party, the European MS Platform gladly acknowledges a wonderful cooperation with EMA on preparation, running and follow up of its workshop on MS Registries in Europe.



Christoph Thalheim (EMSP)

Digital media is also being used in following the safety of medicines, as patients are providing a wealth of information to each other via social media and initiatives such as [WEBrADR](#), where patients and regulators have been extensively involved, which not only aim to mine the text of public data but also to develop an application as a platform for patients and healthcare professionals to access accurate and timely information.

A two-day [workshop on data anonymisation](#) covered the topics of clinical trial data, individual patient level data and real world data in the context of patient registries and individual cohort studies. The workshop brought together key stakeholders from patient organisations, academia and healthcare amongst others. Representatives from EURORDIS, GAMIAN Europe and eYPAGnet³ spoke on behalf of the patient community during their session on "Defining sensitive data – influence of the context of the disease on the tolerability of risk".

³ eYPAGnet - European Young People's Advisory Group network

2. Interactions with patients and consumers

2.1 Introduction

As with previous years, patient and consumer involvement has consistently increased reaching a total of 925 cases in 2017 (Figure 2).

This year saw the first public hearing being held, a landmark event that brought together patients, consumers, healthcare professionals, pharmaceutical industry and the public to provide their experience to complement the scientific evidence in the evaluation of valproate-containing medicines. This is another string to the bow of methodologies for public engagement at EMA.

With the adoption of the EMA's "Principles on the involvement of young patients/consumers within EMA activities", EMA is opening the doors for inclusion of the voices of young people in medicines' evaluation. Understanding that training and support is needed, young people participated in the annual training day for the first time in 2017.

Regular support and outreach towards our network of 102 registered patient and consumer stakeholder organisations was maintained and they were kept informed of EMA activities, public consultations and safety communications via our stakeholders' database throughout the year. In addition, the individual expert database, launched in 2016, continues to grow and currently has more than 380 registered individuals. The database is used to share relevant information as well as to invite individuals to participate in EMA activities in their areas of interest.

As always, the eligible patient and consumer organisations remain the first line when EMA is seeking to involve patients in its activities. Representatives from some of these organisations are also members of the PCWP.

“

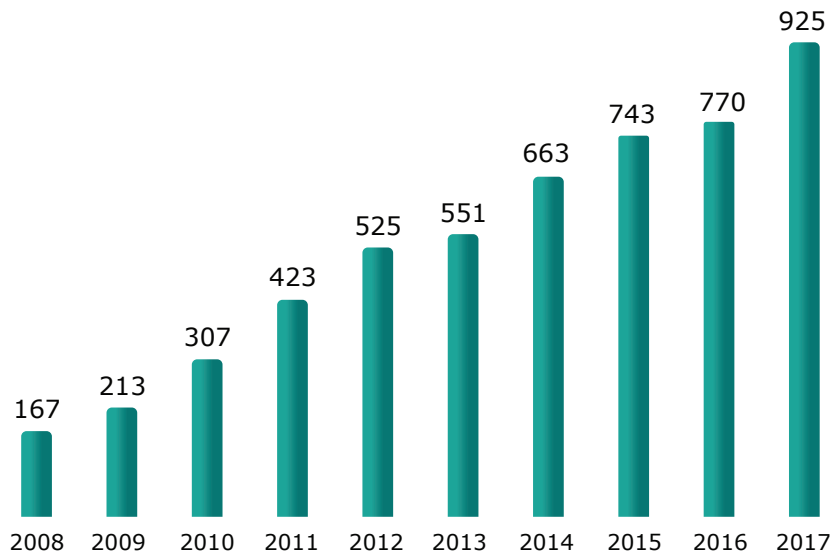
In 2017, the CHMP decision to continue inviting patients at oral explanations with the applicant, based on the outcomes of the 2-year pilot, was an important milestone. For patients' representatives, witnessing how CHMP adopts opinions and contributing to their scientific discussions on delicate dossiers is key. This illustrates the fact that patients are considered with equal credibility as other experts.

The first Public Hearing on pharmacovigilance was another major achievement, and a great success, with patients providing their experience and suggesting new measures to minimise risks when using valproate sodium during pregnancy. A first event that holds promises for future ones called for by the PRAC and, who knows, other committees?

” **François Hoüyez (EURORDIS)**

In summary, this chapter describes how patients and consumers have participated in EMA activities in 2017. More information on how patients and consumers can be involved in EMA activities is available on our [website](#).

Figure 2. Overall number of patient and consumer involvement in EMA activities (2008-2017)

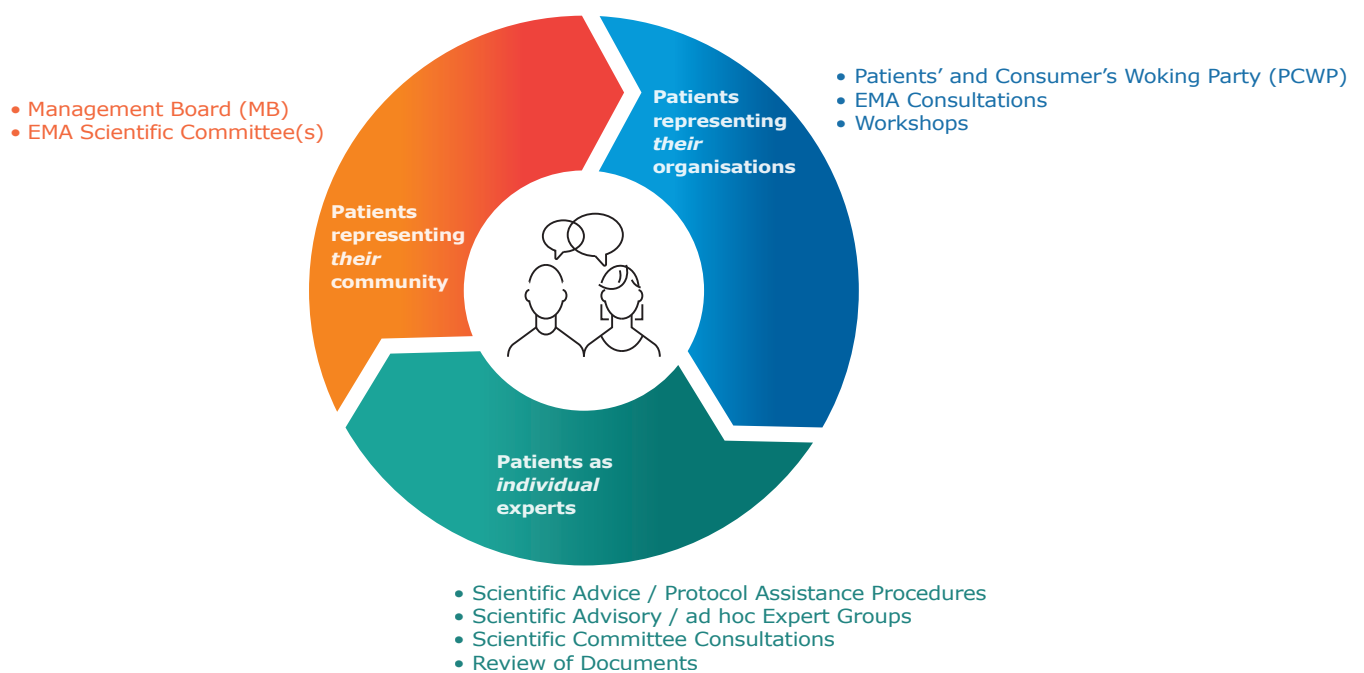


2.2 Patients/consumers in EMA activities and scope of representation

When patients and consumers are invited to participate in EMA activities, the capacity in which they do so depends on the activity concerned. In Figure 3, we see the broad representation of those involved in scientific committees or in the Management Board where their role is to represent

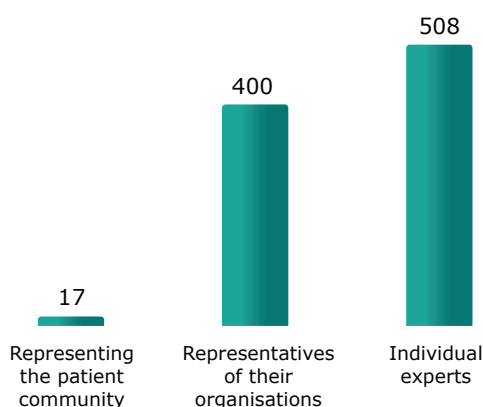
the patient community in the EU. In other activities, patients represent their own organisation and in the case of medicine-related activities such as scientific meetings, consultations by committees or review of documents, they act as individuals.

Figure 3. Patients and consumers in EMA activities and scope of representation



In Figure 4, the numbers of patients involved in the categories described above for 2017 are shown. More detail about each of these activities is provided in the corresponding sections below. For an overview of patient involvement in EMA activities since 2007, please see Annex I.

Figure 4. Overview of involvement in EMA activities, by type of representation (2017)



2.3 Patients/consumers representing the community

Membership in EMA management board and scientific committees

Patients and consumers are members of the EMA management board as well as the COMP, PDCO, CAT and PRAC⁴ (Annex IIA). Patients are also invited on a case-by-case basis to contribute to specific medicine discussion, for example they regularly take part in oral explanations at the COMP and more recently, the CHMP, following a successful pilot project in 2016 (Section 2.5.1.3).

Support to committee members representing patients

In addition to the mandated tasks, the role of these committee members is to represent the broader patient community in Europe and bring that perspective to the committee discussions. Regular meetings have been organised by the Public Engagement department to ensure that these members have the support they need and to assist them when they need to consult other patients on issues within committee evaluations. In addition, the Committees department is exploring methods for facilitating and supporting their committee work as these members are not part of any national competent authority.



⁴ COMP – Committee for Orphan Medicinal Products;
PDCO – Paediatric Committee;
CAT – Committee for Advanced Therapies;
PRAC – Pharmacovigilance Risk Assessment Committee

2.4 Patients/consumers representing their organisations

2.4.1 Membership of Patients' and Consumers' Working Party (PCWP)

The Patients' and Consumers' Working Party ([PCWP](#)) is composed of 20 members and 21 alternates (Figure 5) from 20 selected organisations that fulfill

EMA's eligibility criteria. The working party is co-chaired by a member of the working party, Kaisa Immonen (EPF), and a representative from EMA, Juan Garcia Burgos. Patient representatives are also observers on the Healthcare Professionals Working Party (HCPWP).

Figure 5. The Patients' and Consumers' Working Party (PCWP)



The PCWP collaborates and holds common meetings with the Healthcare Professionals Working Party (HCPWP). In 2017, joint meetings were held in [March](#), [June](#), and [September](#).

In addition, the annual meeting with all EMA eligible patient and consumer organisations was held on [22 November](#) and discussions included the public hearing on valproate, the EMA action plan to improve product information for EU medicines and the Agency's use of new communication materials to better engage the general public. Participants were also updated on big data and real world evidence including information on the acceptability of big data for regulatory decision making. Two organisations, EUomo and EURORDIS, gave presentations as part of the "Members' Voice" section.

The team at EMA was saddened by the loss of Christine Lavery (FIN) in 2017. She will be remembered for her dedication and tireless work for patients.

“ EHN is keen on bringing the voice of patients living with cardiovascular diseases to the EMA regulators and is particularly pleased to realise that their voice is actually heard! We've also seen great impact coming out of the exchange between the EMA and the eligible organisations of the Patients and Consumers Working Party (PCWP). We value the openness of the discussions, the exchange of best practices and variety of insights we gather at the PCWP meetings and work - truly useful and pertinent.

” **Sofia Marcha (EHN)**

2.4.1.1 Topic groups of the PCWP

Topic groups remain an important working party activity enabling the continuation of discussion outside the margins of the plenary meetings. They are a means to facilitate collaboration in smaller groups on specific areas and to include other experts from the organisations. Two PCWP topic groups were ongoing in 2017; Involvement of young people in EMA activities, which has resulted in the adoption in July of the [Principles on the involvement of young patients/consumers within EMA activities](#) by the EMA Management Board. The principles define how young people could contribute and suggest options on how best to capture their opinions.

The second topic group, entitled “Digital Media and Health” has evolved from the previous topic group on social media. This group covers issues around social media, mHealth and real world evidence. The outcomes of these topic groups will be published once finalised.

2.4.2 Workshops, meetings and consultations

An overview of the activities where patients and consumers were involved representing their own organisation are listed in Table 1. These include participation in workshops and conferences, working party related activities, consultations with

committees as well as meetings with patient groups to explore the patient preferences project or to discuss how they as an organisation can engage further with EMA.



The involvement of the civil society in the EMA’s work can be considered a model to be applied at national level. We would like to thank the PCWP because we always felt that our contribution is very well received, welcomed and valued by the Agency. Along the year we not only participate in meetings but also provide input in public consultations and to revise information about medicines that are provided to the patients, namely package leaflets and safety communications.



Joao Nabais (APDP)

Table 1. EMA Activities involving patient and consumer organisations

Activities involving patients’ and consumers’ organisations	Number of representatives
Workshops and conferences	
Patient participation in workshops at EMA	138
PCWP - related activities	
PCWP members and alternates (membership)	42
PCWP Topic Groups – calls with Involving Young People/Digital Media and Health groups	13
Drafting group for the PCWP-HCPWP work plan for 2018-2019	3
Observer at HCPWP meetings	1
Ad-hoc observers/experts attending PCWP meetings	14
PCWP meeting with all eligible organisations	36

Activities involving patients' and consumers' organisations	Number of representatives
Renewal of eligibility status for eligible organisations	36
Support and information to patients' and consumers' organisations	
Meeting with European Multiple Sclerosis Platform (EMSP)	1
Meeting on medicine availability with Myeloma Patients Europe (MPE)	1
Information meeting with patients on how to get involved with EMA:	
- EU-IPFF (idiopathic pulmonary fibrosis)	4
- Loulou Fondation (CDKL5 deficiency disorder)	1
- Muscular Dystrophy UK	1
Meeting with Pulmonary Hypertension Europe (PHA) for workshop participation	1
Expanding patient input: exploring new methodologies	
Patient Preference elicitation studies:	
- Meetings and teleconferences with Alzheimer Research UK	8
- Meeting with patient from Myeloma Patients Europe (MPE)	1
- Meeting with Parkinson's UK	1
Interactions with scientific committees	
PRAC Stakeholders Meeting on Valproate	11
PRAC Stakeholders Meeting on Retinoids	7
PRAC written consultation on Valproate	9
PRAC written consultation on Dopamine dysregulation syndrome	3
PRAC written consultation on medicine for hypothyroidism	2
CHMP/PRAC annual meeting with Thalidomide UK	1
CHMP written consultation- Reflection paper on frailty	2
CHMP written consultation- new medicine for treatment of neuroblastoma	3
HMPC plenary meetings - observers	7
PRAC Public Hearing	
Public Hearing on Valproate	20
Support to members representing patients on scientific committees	
Annual meeting with civil society representatives of scientific committees	9
Support and information meetings	22
Collaboration with FDA	
EMA/FDA Rare Diseases Cluster: medicine for the treatment of a rare disease	1
Gaucher disease: an EMA/FDA strategic collaborative document	1
Total	400

More information on workshops can be found in the annexes, including a complete list of workshops in 2017 (Annex IV) as well as an overview of patient participation in workshops since 2007 (Annex V). More workshops are listed in Section 1.5.

Public-private collaborations and partnerships for vaccine benefit-risk monitoring in Europe: the ADVANCE framework and governance principles

A joint two-day workshop between EMA and Accelerated Development of VAccine beNefit-risk Collaboration in Europe (ADVANCE) on co-ordinating vaccine benefit/risk monitoring across Europe was held in March. The aim of this workshop was to address how a public-private collaboration or partnership for vaccine benefit-risk monitoring in Europe could be set up and how the various stakeholders could work together in full transparency. Participants included representatives from national public health institutes and ECDC⁵, national competent authorities, academia and industry. The patients' and consumers' communities were represented at the workshop by attendees from BEUC, ECPC, EIWH and IPOPI. A summary of the workshop is available [here](#).

2.4.2.1 Expanding patient input: exploring new methodologies

Elicitation of patient preferences and values on benefits and risks projects

Following the patient preference study in 2016 involving multiple myeloma, EMA is exploring the feasibility of conducting similar studies in other disease areas such as Alzheimer's and MS and is currently collaborating with Alzheimer's UK on another such study.

EMA also collaborates with the [IMI-PREFER](#) project, a consortium of stakeholders who over the next five years will run patient preference studies in both academic and industry settings. The consortium is working to establish recommendations to support the development of guidelines for industry, regulatory authorities and HTA bodies on how and when to include patient perspectives on benefits and risks of medicinal products. These efforts will further help to place patients at the centre of decision making throughout the life cycle of medicines.



⁵ ECDC – European Centre for Disease Control

2.5 Patients and consumers as individual experts

When patients and consumers are involved in EMA activities on medicine-specific issues, they do so as individual experts. Table 2 provides an overview of the activities and number of patients and consumers involved. Some of these activities are

described in more detail below. Patients attended the public hearing both as individuals and also as representatives of their organisation, which is reflected both here and in Table 1.

Table 2. EMA activities involving patients and consumers acting as individual experts

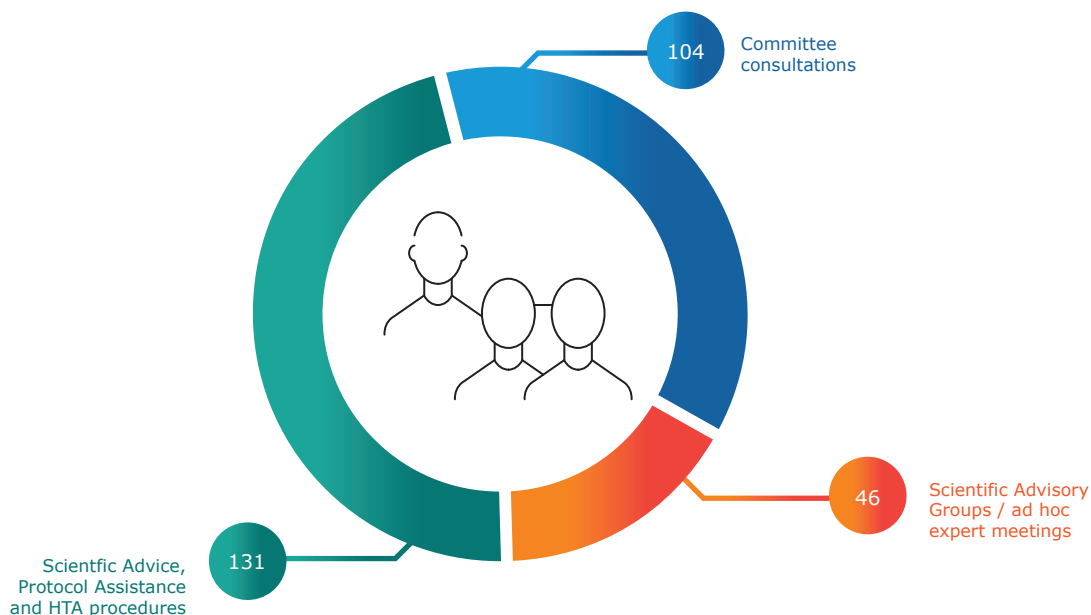
Activities involving individual experts	Number of Experts
Review of documents	
Herbal summaries	34
EPAR summaries	39
Package Leaflets	80
Safety communications (CHMP)	8
Safety communications (PRAC)	13
Safety communications (EMA)	3
Involvement in medicines evaluation / committee consultations	
COMP oral explanations	6
CHMP oral explanations	4
PRAC written consultation- Risk minimisation of medication errors	15
PRAC/QRD written consultations on the PL and pack design for two medicines for the treatment of diabetes	9
Scientific advice	80
Protocol assistance for orphan medicines	33
Scientific advice/protocol assistance with health technology assessment bodies	18
Participation in Scientific Advisory Groups (SAGs)/Ad hoc expert group meetings	46
Public Hearing	
Public Hearing on Valproate	8
Training	
EMA annual training day	56
Webinar: Herbal medicines	4
Webinar: Review of EPAR summaries and package leaflets	52
Total	508

2.5.1 Patient and consumer involvement in scientific meetings

Figure 6 provides an overview of individual expert patient involvement in scientific procedures such as scientific advice (SA), protocol assistance (PA), scientific advisory groups (SAG) and ad hoc expert

group meetings as well as consultations by scientific committees (CHMP/PRAC). Further details on each of these activities is provided below.

Figure 6. Patient and consumer involvement in EMA activities (2017)



As discussed in Section 1.6. the Agency continued to collaborate closely with health technology assessment (HTA) bodies and in 2017 18 patients participated in

parallel scientific advice/HTA procedures. This figure has been increasing steadily since patients were first involved in 2012.

“Members of the European Society of Endocrinology and, more importantly, European patients with endocrine disorders benefit directly from the activities of the European Medicines Agency. In addition to evaluating applications for marketing authorisation and the monitoring of medicine safety, the EMA provides essential advice for the development and approval of pharmaceutical compounds, creates accessible patient information on drug safety and efficacy, and implements regulatory mechanisms to improve access to new medicines. EMA initiatives, such as the incentives offered for orphan medicines, likely have direct, positive impacts on endocrine healthcare in the EU.

” **Alex Harrison (ESE)**

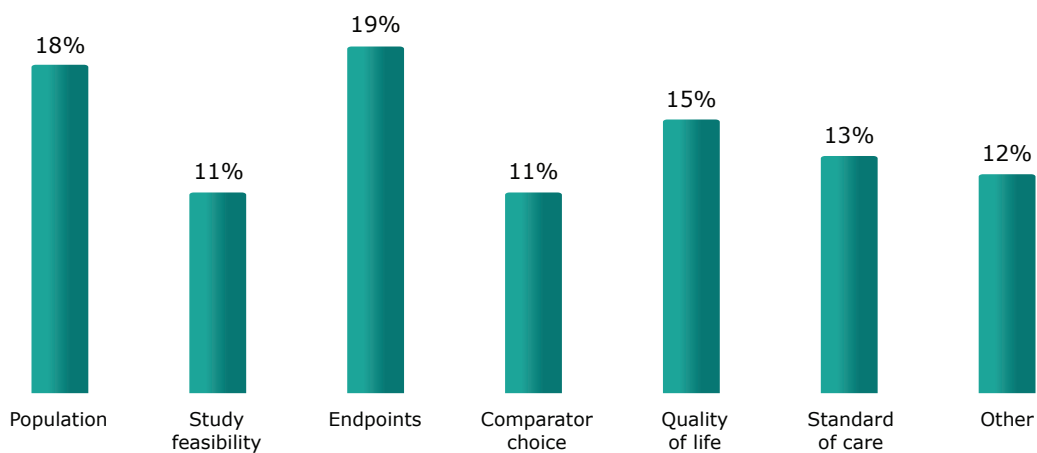
2.5.1.1 Input into scientific advice (SA) / protocol assistance (PA) procedures

Patient input into scientific advice is an example of patient engagement in early dialogue in medicines development. When a company requests scientific advice, they can ask questions on all aspects of development of a medicine from its manufacture to preclinical studies and clinical trials. In 2017, 131 patients were involved in SA, PA and HTA procedures

at EMA. Patients participated both in writing and in person at discussion meetings with medicines developers.

A new survey was launched with the EMA scientific officers regarding patient participation in scientific advice processes and feedback (90 responses received) provided highlights of the particular elements that patients commented on and the added value of their participation in this activity.

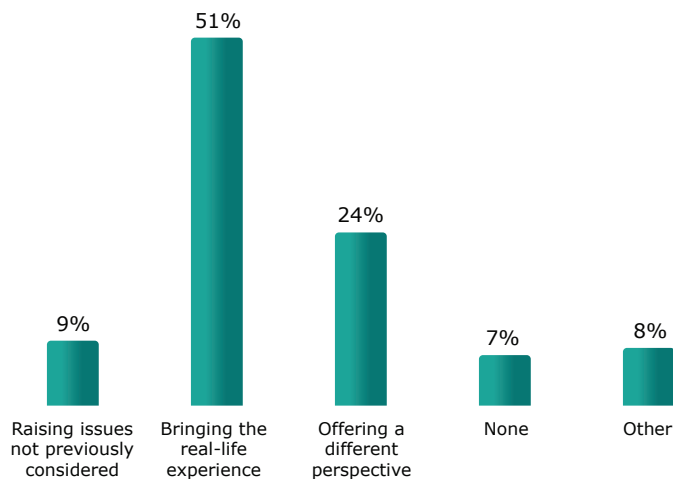
Figure 7. Aspects of development plan where patients gave most input



Patients most frequently provided input on issues such as study population, endpoints of the study and quality of life (Figure 7). Other aspects mentioned

included study duration, safety concerns and more specific issues related to the particular condition.

Figure 8. Added-value of patient input



In addition, scientific officers felt that patients brought the real life experience into the process as well as offering a different perspective to the other evidence presented (Figure 8). An important consideration that is sometimes overlooked when considering the 'impact' of patient involvement in scientific procedures is agreement with the development plan and the 2017 survey shows that nearly 80% of patients agreed with the advice given by the Scientific Advice Working Party (SAWP) to the company. Comments, when provided by patients, resulted in further reflection by the coordinators of the procedures in 53% of cases and a further 20% of patients agreed with the proposed responses to the company and therefore did not make additional input in the final advice letter.

In one example, a patient provided comments about patient variability and study safety for medicine being developed to treat an autoimmune disease. The patient also highlighted specifically the signs and symptoms of adverse events that patients need to be made aware of. The patient's valuable input was incorporated into the final advice letter sent to the company.

In another example with an orphan medicine, the patient's real life knowledge of the condition allowed them to advise coordinators on the significance of the endpoints of the proposed clinical trial. This resulted in further reflection by the Scientific Advice Working Party and the patient's input was incorporated into the final advice letter.

“ Participating in EMA meetings provides a greater understanding of the many stakeholders involved in the process of approving medication, and how patient representatives are engaged, to ensure that their experiences and concerns are included in the discussions. It is encouraging to see, at first-hand, that the voice of the patient is on the EMA agenda.

” **Ann Little (IBE)**

The total numbers of patients involved in scientific advice procedures since 2009 is available in Annex VI. To learn more about scientific advice and how patients can be involved please see the following [document](#), [video](#) and accompanying [presentation](#).

2.5.1.2 Input into Scientific Advisory Groups (SAG)/ad hoc expert meetings

Patients are invited to participate in all scientific advisory groups and ad-hoc expert meetings convened by the CHMP or PRAC. These meetings are held to provide advice in connection with the evaluation of a particular medicine or treatment. During 2017, 24 SAG/ad-hoc meetings involving 46 patients were held and covered a range of therapeutic areas. The input of the patients is appreciated by other members of the advisory groups and reflected in the minutes; examples below highlight the important contributions that patients make within these meetings.

In a SAG for a medicine authorised for multiple sclerosis, the discussion was focused on review of the risk that had been identified at time of marketing authorisation and addressed in the risk minimisation plan. The PRAC, the EMA safety committee, convened the SAG, considered that provisional measures were needed while the issue was being further reviewed. One of the patient representatives recommended that the possibility of changing to a different treatment should be communicated to patients before they start treatment. This was supported by other members of the SAG.

While the second patient representative considered that this particular medicine was not particularly suitable as a first treatment option, based on personal experience, they were of the opinion that this medicine should be available for people that have no alternatives and emphasised the importance of informing patients of the risk for them to be able to make informed treatment decisions.

In two separate SAGs convened by the CHMP; in both cases the patient representatives emphasised that more robust data generation and definition of the patient population that would benefit most from the medicine would be welcome as trying several ineffective medicines before finding an effective treatment is a major burden.



Just a note saying thank you for an excellent draft report. I am really pleased that both of our remarks (patients) were taken into the consideration in this draft. Thank you for the opportunity to be a part of this meeting.



Patient involved in SAG meeting

2.5.1.3 Scientific committee consultations

Scientific committees consult with patients either by inviting them to the plenary sessions or by written consultations. In 2017 the PRAC consulted with patients on five different issues; one instance that has already been described was the public hearing on valproate containing medicines and others include contributions to a survey on reporting adverse reactions for medicines subject to additional monitoring [report to follow] as well as providing input on how to minimise risks associated with withdrawing insulin from pre-filled pens and cartridges. This latter consultation resulted in the PRAC recommending the inclusion of new warnings in the summary of product characteristics document (SmPC) and package leaflets of these products.

Patients were also involved in two consultations with the QRD team (quality review of documents) regarding packaging warnings to reduce the risk of errors. The first concerned the acceptability of the packaging proposed for an insulin combination and existing initiatives of using labelling and colour coding to reduce medication errors with antidiabetic medicines. The second was regarding the expression of strength, composition and posology for a cancer medicine.

Patient involvement at the CHMP

In May 2017, the agency published a final report on the experience gained during the pilot project to involve patients in oral explanations at the CHMP. During the pilot, patients participated in discussions at the CHMP and gave their views on the benefits and risks of six medicines. The feedback from CHMP members and patients was positive and confirmed the benefit of including patients in discussions at the CHMP when the patient perspective could complement the assessment.

The report concluded that patients should continue to be invited to oral explanations on a case-by-case basis. The final outcome report on the pilot project is available [here](#). An analysis of the pilot can be found in the [2016 public engagement annual report](#).

2.5.2 Review of EMA information

In the context of transparency, the EMA makes this information on the various aspects of a medicine's evaluation public via its website and also generates documents that are tailored to patients that are reviewed by patients and consumers to ensure they are clear and can be understood. Patients and consumers routinely review package leaflets (PL), medicines overviews formerly known as EPAR summaries, safety communications and more recently herbal summaries.

Documents reviewed by patients

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.

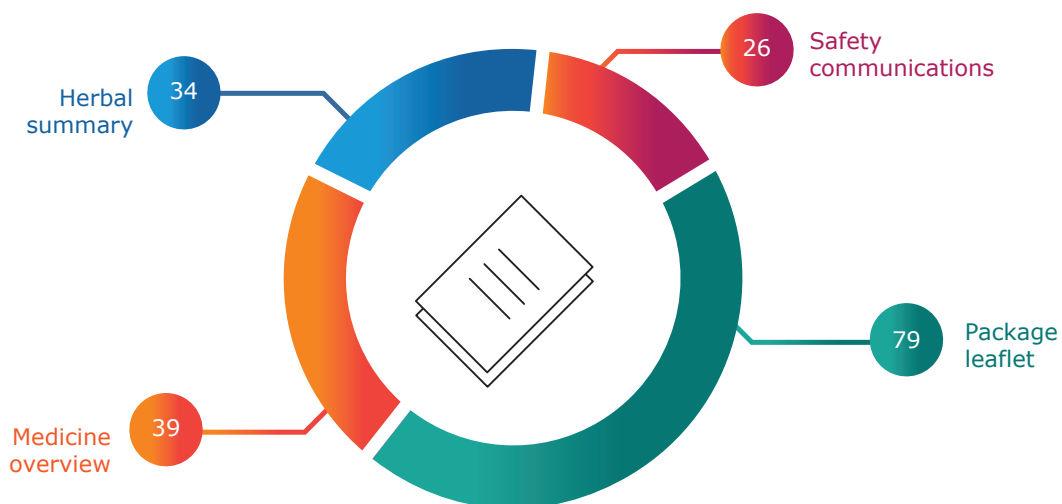
Medicine overviews are lay-language documents that provide a summary of the grounds on which the CHMP based its recommendation for the medicine to receive a marketing authorisation.

Safety communications refer to documents that are specifically addressed to the public on authorised medicinal products and that convey an important (emerging) message relating to the product (e.g. a product is withdrawn or suspended for safety reasons, has a new contraindication or warning, or there is a product defect).

Herbal summaries are summaries of the scientific conclusions reached by the Committee on Herbal Medicinal Products (HMPC) on the medicinal uses of herbal medicines. The HMPC conclusions are taken into account by EU Member States when evaluating applications for the licensing of herbal medicine.

In Figure 9, the number of documents reviewed by patients and consumers in 2017 is shown. For the number of documents reviewed since 2007 please see Annex VII.

Figure 9. Documents reviewed by patients



2.6 Capacity-building and awareness-raising activities

Capacity-building 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.

Capacity-building activities

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.

Annual training day: A training day is organised that introduces EMA to stakeholders and invites them to participate in hands-on breakout sessions on i) scientific advice, ii) Scientific Advisory Groups and iii) review of documents.

EMA Annual training day for patients and healthcare professionals
EMA provides training and support to ensure the voice of patients and healthcare professionals is heard all along the regulatory lifecycle of medicines.

About
EMA's annual training day is one of many engagement methodologies and resources used to support the involvement of stakeholders in its work. This initiative was first started for patients and consumers in 2007.
Participants learn about EMA, medicines evaluation and practice by participating in examples of real procedures
In 2017, healthcare professionals and young people were included in the training day for the first time
Based on feedback received and regulatory updates, the content and format of training day is adapted each year

Objectives
Understand how medicines are authorised and monitored in Europe
Appreciate where and how patients and healthcare professionals can use a hands-on approach
The topics covered are:
Scientific Advice
Scientific Advisory Groups
Document review (Medicines overview and safety communications)

Outcomes
A total of 397 participants trained to date.
Trained participants have subsequently become members of:
Management Board
Scientific committees
Working parties
Have been invited as experts in:
Scientific advice
Scientific advisory groups
Review of documents
Written consultations

Overview
2017 - 71 participants
2007 - 2017
52 patients and consumers
13 healthcare professionals
4 young people
3 academics

Additional resources
Videos - EMA's bite-sized introductory information on YouTube:
EMA Basics Collage
How are medicines approved?
Stronger medicines rules

EMA has also explored the use of webinars as an educational and communication tool. Two webinars were held in 2017; one on review of herbal summaries and the other on review of medicines overviews and package leaflets.

Webinars were also used for the annual training day at EMA to provide participants with an overview of both logistics and content of the training.

The training day in 2017 included a total of 68 participants (52 patients, 13 healthcare professionals and 3 academics) representing 20 different EU countries. This brings the total number of patients trained at EMA to 378 (since 2007). Healthcare professionals were included for the first time in 2017 (for more information, please see Section 3.6).

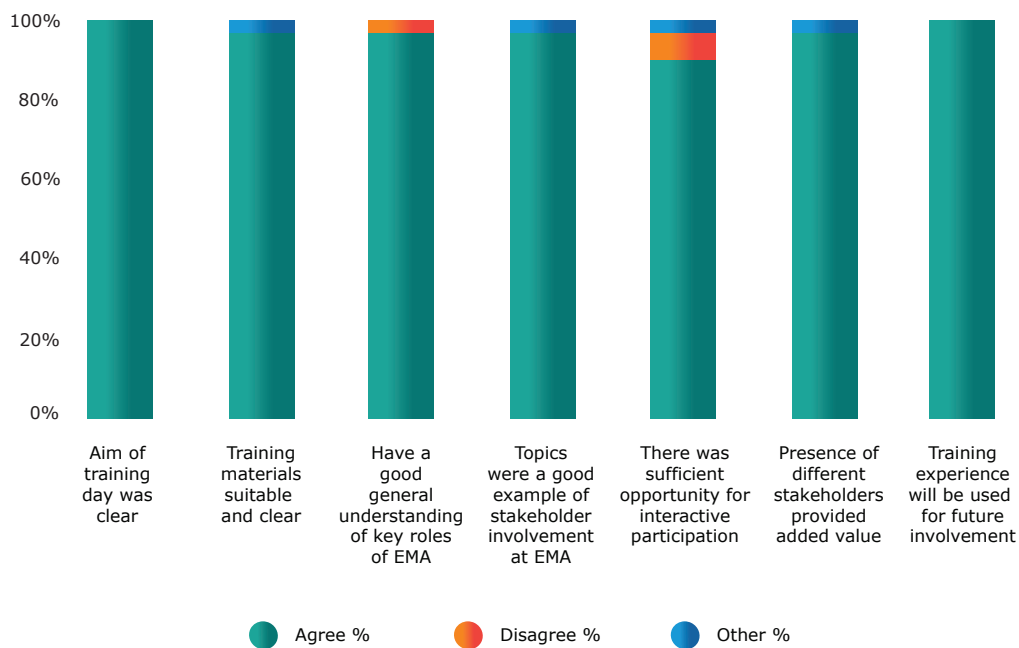
“

We were particularly impressed by the Training Day, where we were able to send some of our young representatives to learn about the work of the EMA (which they found very informative). It is also very welcome to see true user involvement in the various consultations carried out in between the PCWP meetings. This indicates that EMA truly is interested in listening to, and acting upon, the voice of the patient and takes our concerns seriously. We look forward to continuing our engagement with the EMA as they begin to make their move from London to Amsterdam.

”

Simon O'Neill (Diabetes UK)

Figure 10. Feedback on annual training day from patients and consumers



Four representatives from European YPAG’s (Young Persons’ Advisory Group) network took part in the training for the first time and their feedback will help us tailor support and training for the involvement of young participants. Specific training material needs to be developed to ensure appropriate support for their involvement.

For more information on training provided to patients and consumers please visit our [website](#).

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 activities that raise awareness regarding patient engagement and has participated in meetings organised by external stakeholders.



2.7 Organisations involved in EMA activities during 2017

There are currently 34 eligible patients' and consumers' organisations (see Annex VIII). These are the organisations with whom the Agency collaborates on a regular basis and who are usually the first to be involved in stakeholder consultations.

This list is published on the Agency [website](#) and includes links to their websites and a summary of their mission and objectives. All eligible organisations have been evaluated and confirmed to fulfil the EMA eligibility criteria. More information on the eligibility criteria and engaging with EMA representing a patients' or consumers' organisation is available on our [website](#).

Figure 11. Eligible patients' and consumers' organisations working with the EMA in 2017



In addition to the eligible organisations, 59 other patients' and consumers' organisations also interacted with the Agency and are listed in Table 3.

These organisations provided experts for scientific advice and SAG meetings or they contributed to consultations or participated in workshops or conferences.

Table 3. Organisations consulted by EMA on specific areas

Organisations consulted by the EMA on specific areas	
Acquired aplastic anaemia (AA) and paroxysmal nocturnal haemoglobinuria (PNH) Contact group	FOP French Association
ALPE Achondroplasia Foundation	Fundacja Sanfilippo Poland
Amyotrophic lateral sclerosis (ALS Liga) Belgium	Gauchers Association
Associação de Jovens Diabéticos de Portugal	Hepactive patient association - Bulgaria
Association for Glycogen Storage Disease (UK)	Independent Fetal Anti Convulsant Trust (In-FACT)
Association of Diabetics of Portugal	International Prader-Willi Syndrome Organisation
Association of Parents of Children with the syndrome anticonvulsant (APESAC)	Italian Association for Laryngectomy Patients
Association Suisse du Syndrome de l'Anti-Convulsivant (ASSAC)	Lupus UK
Belgian Association of Victims of Valproate Syndrome (ABVSV/ BVSVS)	Lymfoma Slovakia
Bipolar UK	Lymphoma Coalition Europe
Bowel Cancer UK	MDS UK Patient Support Group/MDS Alliance
British Heart Foundation	Melletted a helyem Egyesulet – Hungary
Brother France SAS	Mitocon Onlus Italy
Bulgarian Society of Patients with Pulmonary Hypertension	Myasthenia Gravis Association UK
Croatian Association for Epilepsy	Narcolepsy UK
Cystic Fibrosis Europe	NET - group Foundation
Deutsche Leukaemie- & Lymphom-Hilfe e.V.	Niemann-Pick Disease Group (UK)
Diabetes UK	OACS Ireland & FACS Forum Ireland
Drug Induced Mitochondrial Toxicity (DIMITOX)	Parkinson's UK
Epilepsiforeningen	Polycystic Kidney Disease Charity (PDK)
Epilepsy Action	Prader-Willi Syndrome Romania
Epilepsy Society	Sickle Cell and Thalassaemia Ireland
Epilepsy Ireland	Society for Mucopolysaccharide Diseases
Europa Donna	The British Kidney Patient Association
European Idiopathic Pulmonary Fibrosis & Related Disorders Federation	The Czech CF Association (CCFA)
FACSAware	The European Umbrella Organisation for Psoriasis Movements (Europso)
Federal Association of short stature people and their families Germany	Vereniging voor Kinderen met Stofwisselingsziekten
Federation of European Psoriasis Association	Vereniging voor Mensen met Constitutioneel Eczeem (VMCE)
Fetal Anti Convulsant Syndrome Association (FACSA)	Wolfram Syndrome UK
Fighting Blindness	

2.8 Exchange of practices of patient engagement

2.8.1 US Food and Drug Administration (FDA)

The EMA/FDA cluster on patient engagement continued in 2017. The cluster is a working group led by EMA's Public Engagement Department, together with Patient Affairs Staff (PAS) in the Office of Medical Products and Tobacco (OMPT). Three cluster teleconference meetings were held in 2017, providing a forum for sharing experiences and best practices on how the two agencies involve patients in development, evaluation and post-authorisation activities related to medicines.

Both agencies consider the involvement of patients to be essential and areas of discussion have included the processes for selecting and preparing patients to take part in the agencies' activities, how to ensure that patients are independent and representative, and how to report on the impact of patient involvement.



3. Interactions with healthcare professionals

3.1 Introduction

As with previous years, the involvement of healthcare professionals in EMA activities increased in 2017 with a total of 445 cases (Figure 12).

Different medical specialists, pharmacists and nurses provided valuable input in the EMA's first public hearing. This landmark event brought together patients, consumers, healthcare professionals, pharmaceutical industry and the public to provide their experience to complement the scientific evidence in the evaluation of valproate-containing medicines.

Further steps have been made towards more engagement with general practitioners/ family physicians and EMA. Ensuring the input of this first line group of healthcare professionals brings valuable real life input into regulatory procedures and conversely raises awareness of regulatory processes to this group. The European Forum for Primary Care (EFPC), as a member of EMA's working party with healthcare professionals (HCPWP), as well as UEMO and WONCA-Europe, have been instrumental in increasing the input of general practitioners in EMA's activities.

In terms of maintaining our regular support and outreach, 152 registered healthcare professional stakeholder organisations were kept informed of EMA activities, public consultations and safety communications via our stakeholders' database throughout the year.



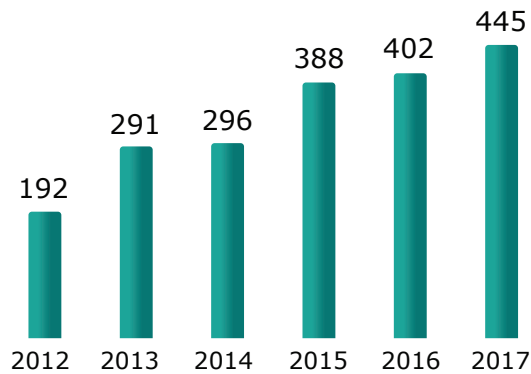
The European Association of Hospitals Pharmacists acknowledges that the on-going engagement of EMA with healthcare professionals, including hospital pharmacists, enables the practical realisation of the benefits and risks associated with medicines to be transferred safely into practice. EAHP actively supports this continued engagement.

 **Stephanie Kohl (EAHP)**

This chapter describes how healthcare professionals have participated in EMA activities in 2017. More information on how healthcare professionals can be involved in EMA activities is available on our [website](#).



Figure 12. Overall number of healthcare professional involvement in EMA activities (2012-2017)



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 the healthcare professional community, representatives of their own organisations or as individual experts. Figure 13 shows the different types of representation

and the corresponding activities. More detail about each of these activities is provided in the corresponding sections below. For an overview of healthcare professional involvement in EMA activities since 2012, please see Annex I.

Figure 13. Healthcare professionals in EMA activities and scope of representation

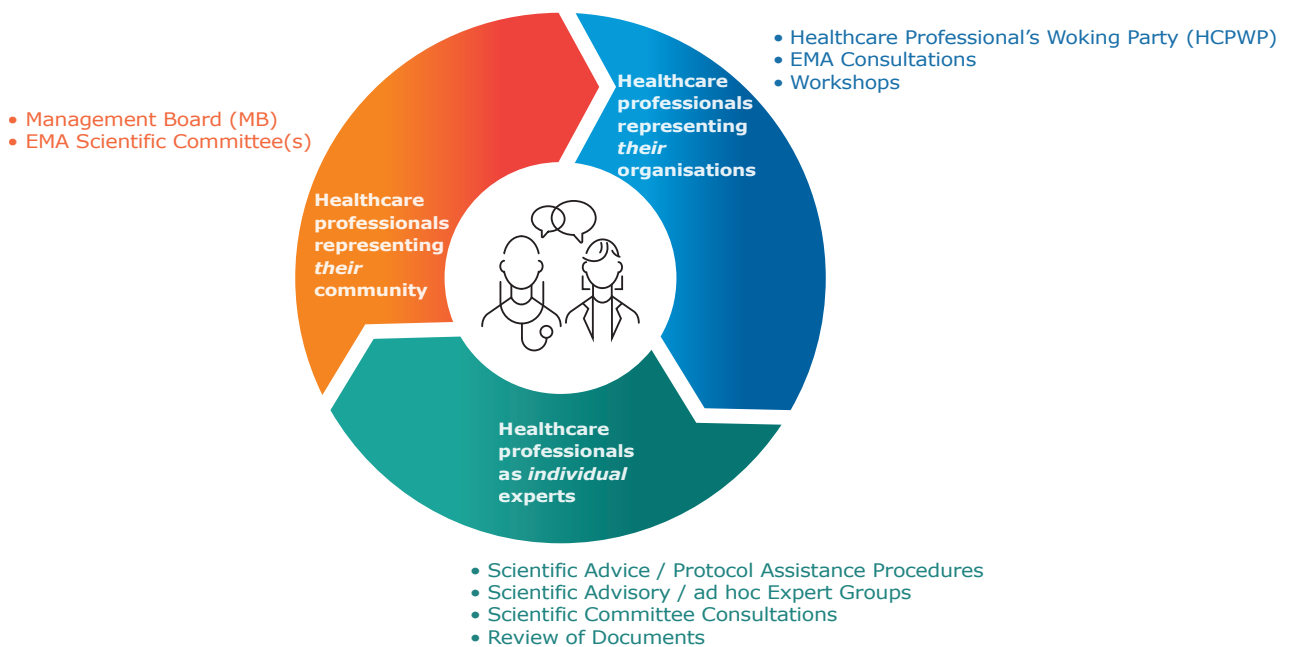
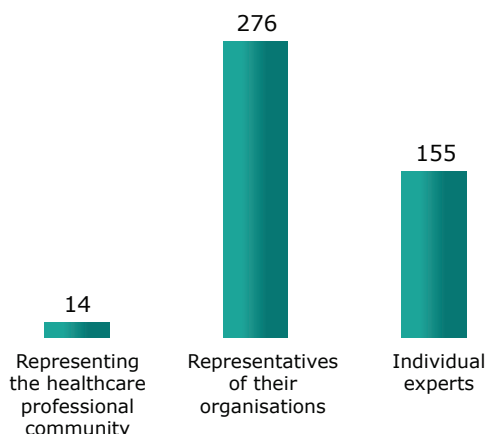


Figure 14 shows the numbers of healthcare professionals involved in the categories described above for 2017. More detail about each of these activities is provided in the corresponding sections below.

For an overview of healthcare professional involvement in EMA activities since 2007, please see Annex I.

Figure 14. Overview of involvement in EMA activities, by type of representation (2017)



3.3 Healthcare professionals representing their community

Membership in EMA management board and scientific committees

As described in Figure 13, healthcare professionals are represented in the [Management Board](#) and in three of the six human scientific committees (Annex IIB). 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.

Support to committee members representing healthcare professionals

These committee members perform the tasks described above and also represent the broader healthcare professional community in Europe and they bring that perspective to the committee discussions. For this reason, regular meetings have been organised by the Public Engagement department to ensure that these members have the support they need and to assist them when they need to consult other healthcare professionals on issues specific to committee evaluations. In addition, the Committees department is exploring methods for facilitating and supporting their committee work as these members are not part of any national medicines agency.



3.4 Healthcare professionals representing their organisations

3.4.1 The Healthcare Professionals Working Party (HCPWP)

The Healthcare Professionals Working Party ([HCPWP](#)) is composed of representatives from 20 selected organisations that fulfil EMA's eligibility criteria

(Figure 15). The working party is co-chaired by a member of the working party, Gonzalo Calvo (EACPT), and a representative from EMA, Juan Garcia Burgos. A healthcare professional representative is also an observer on the Patient and Consumer Working Party (PCWP).

Figure 15. The HealthCare Professionals' Working Party (HCPWP)



The working party met three times in 2017, in [March](#), [June](#) and [September](#), meetings being held jointly with the PCWP. One major topic which was extensively discussed during these meetings was access to medicines (Section 1.6), antimicrobial resistance and biosimilars, the latter being also the subject of a HCPWP topic group.

3.4.1.1 Topic groups of the HCPWP

Some highlights from the topic groups included the conclusion of the Academia topic group with adoption of the [framework of collaboration](#) with academia (for more information, see Chapter 4). The discussion begun with the Risk minimisation measures topic group continued at [the PRAC meeting in May](#) 2017 and resulted in an agreement to focus on developing criteria for triggering consultations with healthcare professionals. The joint HCPWP – PCWP topic group on Social Media was relaunched in May, under the name of Digital Media and Health to reflect its expanded purpose.

EMA would like to acknowledge the sad passing of Reinhold W. Stockbrugger (UEG). He was a valued member of the HCPWP and will be missed.

“

Throughout 2017 the PGEU have been working together closely with the EMA on a number of topics, most notably on the effective implementation and use of risk minimisation measures for a number of medicines commonly supplied through community pharmacies across Europe. Ensuring such measures are practicably implementable, effective and relevant for practice is vital in ensuring patients receive the safest, most rational and effective care.

”

Jamie Wilkinson (PGEU)

In addition to these, a new topic group on Biosimilars emerged whose work contributed to the review of the [EMA/EC guide on biosimilars for healthcare professionals](#).

3.4.2 Workshops, meetings and consultations

The following table details the activities in which healthcare professionals were included as representatives of their organisations in the work of the Agency. It is important to note that the types of activities where healthcare professionals were involved have diversified. These new activities include consultation meetings, focus groups and the public hearing.

Table 4. EMA activities involving healthcare professionals' organisations

Activities involving healthcare professionals' organisations	Number of representatives
Workshops and conferences	
Healthcare professional participation in workshops at EMA	83
HCPWP – related activities	
HCPWP members and alternates	40
HCPWP Topic groups	13
Drafting group for the PCWP-HCPWP work plan for 2018-2019	2
Observer at PCWP Meetings	1
Ad-hoc observers to the HCPWP meetings	7
Renewal of eligibility status for eligible organisations	29
Support and information to healthcare professionals organisations	
Meeting with EURACT regarding EMA interaction with GPs	1
Meeting with UEMO regarding EMA interaction with GPs	3
Meeting with representative from ESNO	1
Interaction with scientific committees	
PRAC stakeholders meeting on retinoids	12
PRAC stakeholders meeting on valproate	14
CHMP Reflection paper on physical frailty	1
PRAC valproate review - Awareness of risks and implementation of risk minimisation activities and their effectiveness	10
PRAC consultation on possible need to raise the awareness of Dopamine dysregulation syndrome amongst patients and healthcare professionals	4
EMA new medicine for neuroblastoma	2
EMA Information guide for healthcare professionals on biosimilars	5
Improving EMA webpages on biosimilars	4
Review of Concept papers on developing/reviewing guidelines	
Quality requirements of medicinal products containing a device component for delivery or use of the medicinal product	1

Activities involving healthcare professionals' organisations	Number of representatives
Evaluation of medicinal products indicated for treatment of influenza	1
Requirements for clinical documentation for orally inhaled products	4
Development and lifecycle of personalised medicines and companion diagnostics	1
Need for a reflection paper on regulatory requirements for development of medicinal products for chronic non-infectious liver diseases	1
Clinical investigation of medicinal products in the treatment of epileptic disorder	4
Role of pharmacokinetics in the development of medicinal products in the paediatric population	3
Focus group	
EMA cloud security consultative group	1
PRAC Public hearing	
Public hearing on Valproate	12
Support to healthcare professional representatives of scientific committees	
Annual meeting with civil society representatives of scientific committees	5
Support and information meetings	11
Total	276

Strengthening collaboration between EMA and general practitioners/family physicians

The dialogue initiated in 2016 with the European Forum for Primary Care (EFPC), the European Union of General Practitioners (UEMO) and the World Organisation of Family Doctors (WONCA) continued over this year.

The ambition of these groups and EMA is to finalise a joint position statement outlining the shared objective and commitment to help EMA gain a better understanding of how medicines are being used in real life and the potential impact of specific regulatory actions on patient care. Further, the aim would be to facilitate the incorporation of views and input from general practitioners in the Agency's activities, recognising the pivotal role that they play in health and patient care and finally to raise awareness amongst this group on the role and activities of the EU medicines regulatory network.



Interaction of the general/family practitioners with EMA is essential for the patient safety as well as for the safe and effective use of medicines. Several ongoing activities concerning rational and safe antibiotic use, vaccine hesitancy and other topics can and will improve the quality of healthcare. This goal can only be achieved by our joint efforts and continuous care.



Nena Kopcavar Gucek (UEMO)

As part of its continued outreach activities, EMA met with the European Academy of Teachers in General Practice/Family Medicine (EURACT) and participated in UEMO's 50th annual meeting.

Overall, the level of involvement of general practitioners in EMA activities was consistent with that of 2016 and included participation in two important stakeholder meetings organised in the context of safety referrals (retinoids and valproate).

Reflection paper on physical frailty

Older persons consume a high number of medicines for chronic diseases, but despite this they have generally been excluded from clinical trials. In 2011, the Committee for Medicinal Products for Human Use (CHMP) requested the Geriatric Expert Group (GEG) to search available documentation and other scientific data to identify available and validated instruments/methods (e.g. scales) to examine effect and safety in "frail" patients. The resulting [reflection paper](#) is the outcome of that work.

There has been interest for some time from members of the HCPWP and the PCWP to develop a topic group for 'older people' however as the GEG existed, it was

considered better to incorporate both patient and healthcare professional voices into the existing group and into this reflection paper.

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

In line with the EMA framework for interaction, healthcare professionals had the opportunity to bring their input and support the activity of the Agency and its scientific committees through participation in written consultations.

Healthcare professionals provided comments on varied issues, from current oncological practices to awareness and educational needs of healthcare professionals in regards to dopamine dysregulation syndrome.

Healthcare professionals greatly contributed to the development of information materials regarding biosimilars targeted at their community. In addition to the information guide mentioned previously, EMA's webpages on [biosimilars](#) were also improved with input from healthcare professionals.

3.5 Healthcare professionals as individual experts

When healthcare professionals are involved in EMA activities on medicine-specific issues, they do so as individual experts.

Table 5 provides an overview of the activities and number of healthcare professionals involved.

Table 5. EMA activities involving healthcare professionals acting as individual experts

Activities involving individual experts	Number of instances
Review of documents	
Safety communications	20
DHPCs (direct healthcare professionals' communications)	13
Involvement in medicines evaluation/ committee consultations	
Participation in SA/SAGs/Ad hoc ⁶ expert group meetings	41
CHMP consultation on oncological product	5
PRAC/ QRD ⁷ consultation on risk minimisation of medication errors with antidiabetic medicine: labelling proposal	6

Activities involving individual experts	Number of instances
PRAC/QRD consultation on risk minimisation of medication errors with antidiabetic medicine: package leaflet and pack design	7
PRAC collected input from healthcare professionals and patients to minimise risk of medication errors associated with use of antidiabetic medicine	15
EMA consultations	
Additional information on the quality comparability data in the European Public Assessment Report (EPAR) for biosimilars	12
Pharmacogenomics in product information	3
Interactions with expert groups	
EMA expert group with general practitioners/ family physicians	9
EMA/FDA strategic Gaucher disease document (registries)	4
Public Hearing	
PRAC public hearing on valproate	7
Training	
Training session for patients, consumers and healthcare professionals	13
Total	155

3.5.1 Healthcare professional involvement in scientific meetings

Through the network of diverse European healthcare professional organisations, the Agency called upon 41 individual experts to participate in 17 different scientific meetings such as scientific advice, SAGs or ad-hoc expert group meetings and bring additional expertise on clinical practice in specific domains during 2017. This expertise was provided on a variety of therapeutic areas and medical fields, such as neurology, haematology and vaccines.

3.5.1.1 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 and facilitate the appropriate and safe use of the medicinal product under assessment. Some examples of consultations are described below.

As part of the initiative to provide better information for healthcare professionals regarding biosimilars, several volunteers were involved in a consultation regarding the inclusion of additional information on the quality comparability data in the European Public Assessment Report (EPAR) for biosimilars. Although it was highlighted that EPARs do not constitute the primary source of information for healthcare professionals, the responses received were generally supportive of the new measures implemented. In addition, the need for more clinical data and information on immunogenicity was noted.

Healthcare professionals were also involved in two QRD (quality review of documents) consultations regarding the use of packaging to reduce the risk of errors. The first one regarded the acceptability of a proposed packaging for an insulin combination and existing initiatives of using labelling and colour coding to reduce medication errors with antidiabetic medicines. The second one concerned the expression of strength, composition and posology for an oncological medicine.

6 SA - Scientific Advice; SAG - Scientific Advisory Group
7 QRD - quality review of documents

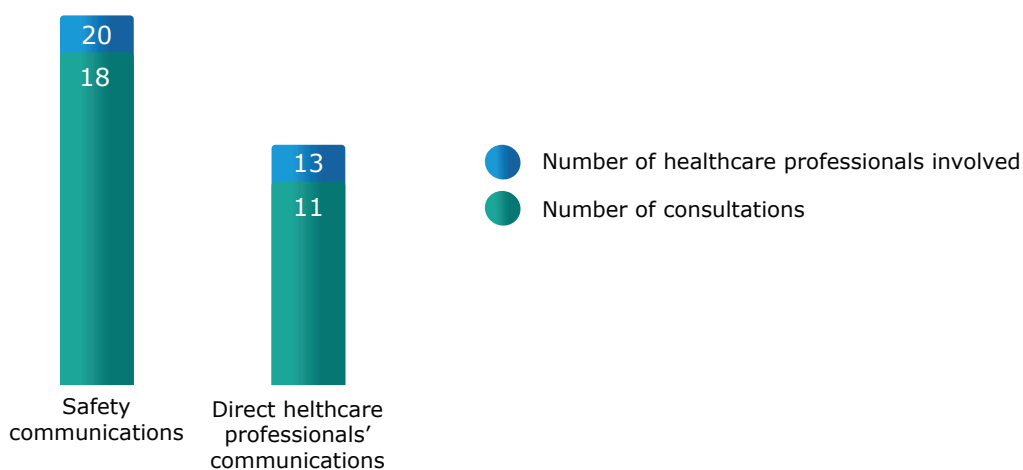
Healthcare professionals also gave their input on ways to minimise risks associated with withdrawing insulin from pre-filled pens and cartridges. The PRAC recommended the inclusion of new warnings in the SmPCs and package leaflets of these products, making them safer to patients all across Europe.

3.5.2 Review of EMA information

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).

Figure 16. Documents reviewed by healthcare professionals



In 2017, a total of 33 experts nominated by healthcare professional organisations (HCPOs) with different specialities and clinical backgrounds were involved in the review of 18 safety communications and 11 DHPCs.

Most of the feedback received was positive with pertinent suggestions used to reinforce the clarity of the messages to be conveyed.

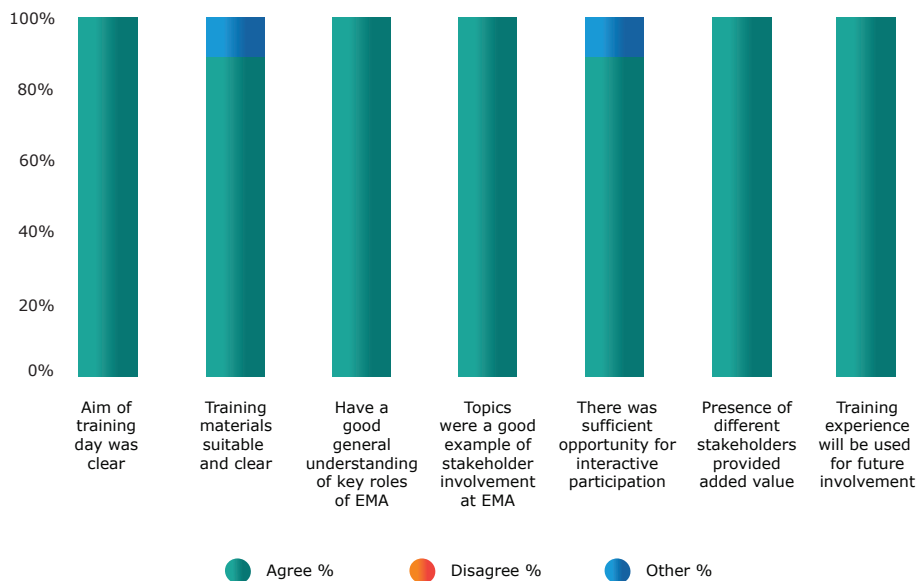
3.6 Capacity building and awareness-raising activities

Capacity building activities

Healthcare professionals participated for the first time in the EMA annual training day, which included a total of 68 participants (13 healthcare professionals, 52 patients and 3 academics) representing 20 different EU countries.

Some of the specialist areas covered by the healthcare professionals included cardiology, paediatrics, community pharmacy, general practice and immunology.

Figure 17. Feedback on annual training day from healthcare professionals



EMA awareness-raising activities

In order to promote further awareness on how the Agency is involving healthcare professionals in its activities, the Agency engages in various activities such as participation in conferences and meetings. In 2017, EMA staff participated in 11 different meetings and conferences organised by healthcare professionals’ organisations.

“ Headache disorders are ranked among the top disabling diseases according to the 2016 Global Burden of Disease Report. The unmet needs in terms of physicians’ education, and awareness of the societal and personal impact of headache shall be pursued through a tight collaboration between the European Headache Federation and the European Medicines Agency. This path will ensure to headache patients a close pharmacovigilance on the risks of the available treatments and a prioritization of ongoing new class drug toward the interception of the chronicisation process.

” **Paolo Martelletti (EHF)**

3.7 Organisations involved in EMA activities in 2017

There are currently 29 eligible healthcare professionals’ organisations (see Annex VIII). These are the organisations with whom the Agency collaborates on a regular basis and who are the first to be involved in stakeholder consultations.

This list is published on the Agency [website](#) and includes links to their websites and a summary of their mission and objectives. All eligible organisations have been evaluated and confirmed to fulfil the eligibility criteria for working with EMA. More information on the eligibility criteria and engaging with EMA representing a patients’ or consumers’ organisation is available on our [website](#).

Figure 18. Eligible healthcare professionals’ organisations working with the EMA in 2017



In addition to the eligible organisations, 13 additional healthcare professionals’ organisations were consulted by the Agency and are listed in Table 6.

These organisations provided experts for scientific advice and SAG meetings or they contributed to consultations or participated in workshops or conferences.

Table 6. Organisations consulted by EMA on specific areas

Name of Organisation	
European Board & College of Obstetrics and Gynaecology (EBCOG)	International Psoriasis Council (IPC)
International Collaboration for Neuroblastoma Research (SIOPEN)	European Society for Paediatric Oncology (SIOPE)
British Dermatological Nursing Group	European Society for Contraception (ESC)
European Network of Teratology Information Services (ENTIS)	World Organisation of Family Doctors (WONCA)
European Psychiatric Association (EPA)	European Reference Network for Rare and Complex Epilepsies (EpiCARE)
European Society for Dermatology and Psychiatry	Foundation of European Nurses in Diabetes (FEND)
British Dermatological Association	

4. Interactions with academia

4.1 Framework of collaboration between the European Medicines Agency and academia

As a science-driven organisation, the European Medicines Agency (EMA) has developed a framework to formalise, structure and further develop interactions with the academic community in the context of the European medicines regulatory network.



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



Guido Rasi (EMA Executive Director)

The development of a coherent framework of collaboration with academia was supported with great interest and commitment from all stakeholders involved and notably by the national competent authorities. After a preparatory phase that began in 2015, the [framework](#) and a supporting [action plan](#) were adopted by EMA's Management Board in March 2017. In-depth discussion with academia and other partners was undertaken to develop the framework considering the importance of striking the right balance between the ambitions, challenges, existing assets and feasibility of a list of actions.

The framework of collaboration with academia will complement existing EMA frameworks that collectively offer a platform of exchange and multi-stakeholder dialogue at the European level. In particular, the framework was conceived to provide a platform to promote dialogue, knowledge exchange on broad scientific issues and advances in medicines development, evaluation and monitoring, clinical research and real world evidence, communication focused on medicines, as well as public and animal health issues and social sciences.



The framework's overall objectives are:

- ▶ raising awareness of the mandate and work of the European medicines regulatory network to increase academia's trust in and engagement with the regulatory system
- ▶ fostering the translation of academic research into novel methodologies and medicines which meet regulatory standards and address the needs of public and animal health
- ▶ ensuring that the best scientific expertise and academic research are available on time to support effective evidence generation, regulatory advice and guidance, as well as decision-making in regulatory processes
- ▶ working with academia to develop regulatory science that embraces scientific progress in medicines development without compromising patient safety, such as for example, the use of novel endpoints or novel methodologies

For the purposes of the framework a definition of academia has been included, although it is recognised that it is a broad and varied stakeholder to define (see framework text for more details). In practice, the Agency will engage with organisations (and their representatives) pursuing education and research in all fields relevant to its work. In particular, the following organisations could fall under the scope of the framework:

- ▶ European research infrastructures;
- ▶ European research consortia funded under public research programmes;
- ▶ European learned/scientific societies, federations and networks.

Under the framework, EMA intends to collaborate primarily with EU-level organisations, although national organisations and networks may be considered in close interaction with the National Competent Authorities (NCAs), which continue to play a central role in managing interactions with academia on their territories.

One important consideration embraced in defining the scope of the framework was that academic research environments are nowadays global and operate at multiple levels and as a consequence consideration will also be given to interaction with organisations beyond the EU territory as and when appropriate.

It should also be noted that the framework of collaboration with academia and the [framework of interaction with healthcare professionals](#) share common objectives with the former focusing primarily on research and education, whilst the latter will continue to focus on clinical practice. Without pre-empting areas of common activity, it is recognised that advances in clinical trial methodology, personalised medicines, novel endpoints, more extensive use of real world evidence generated in the clinical care setting, and the public availability of clinical data will provide additional opportunities for engaging with healthcare professionals and academia at the interface of clinical research and clinical practice.

4.1.1 Preparatory phase to leverage the framework implementation

Following the formal adoption of the framework in March 2017, attention then turned to implementation of the [action plan](#), which included further brainstorming with academic stakeholders and European bodies. This action plan is coherent with activities of the Agency and in particular with the [EMA action plan for small and medium-sized enterprises \(SMEs\)](#). The framework was presented to EMA Scientific Committees and Scientific Coordination Board in order to reflect on how they actively contribute to its implementation. With the same aim, the framework was also presented to the two research networks coordinated by EMA; the European Network of Paediatric Research at EMA ([Enpr-EMA](#)) and the European Network of Centres for Pharmacoepidemiology and Pharmacovigilance ([ENCePP](#)). Overall, the framework was very positively received and appreciation expressed for the activities in the action plan that support its implementation.

The time frame of the action plan spans up to 2019, and annual revisions may be considered taking in account achieved deliverables and the fast changing environment. The main achievements in 2017 included:

- ▶ [Web pages](#) dedicated to academia that provide links to relevant content and a [section](#) that describes how EMA interacts with academia, with more detail on the collaboration framework and action plan and useful resources for academics. Also, a web page dedicated to the European Commission's [Horizon 2020](#) framework programme has also been published in support of researchers applying for funding in the area of health;
- ▶ An EMA entry point on the EMA homepage for academia to receive information on available support within the EU regulatory Network has been made available: academia@ema.europa.eu;

- ▶ The national competent authorities' academia contact points have been identified as the EU-Innovation Offices' representatives;
- ▶ A revised guidance document "[European Medicines Agency process for engaging in external regulatory sciences and process improvement research activities for public and animal health](#)" has been published.

Reaching out to relevant stakeholders will constitute a baseline for the years to come. Concomitant to the framework adoption the first 24 [European Reference Networks \(ERNs\)](#) were launched in March 2017. ERNs are unique and innovative cross-border cooperation platforms between specialists for the diagnosis and treatment of rare or low prevalence complex diseases. They have been set up under the EU Directive on Patients' Rights in Healthcare ([2011/24/EU](#)), and they involve more than 900 highly-specialised healthcare units from over 300 hospitals in 26 EU countries and are working on a range of thematic issues including bone disorders, childhood cancer and immunodeficiency. ERNs stand out as the embodiment of the framework relevant stakeholder, covering clinical research activities, and education and training objectives in addition to the primary patientcare objective. The agency organised an internal information session that provided an overview of the ERNs initiative, delivered by Dr Enrique Terol (Policy Officer - [DG Health and food safety](#) - Directorate B, European Commission). The [session](#) is available on the [EMA YouTube](#) channel. Both EMA and ERNs can benefit from closer collaboration through ERNs providing natural history studies; patient derived outcomes; input into EMA guideline development; provision of expertise from highly specialised clinicians and through EMA providing support e.g via Innovation Task Force, orphan designation, protocol assistance, qualification of biomarkers and methodologies.

In line with the approach taken during the framework preparation we organised informal bilateral meetings were held between EMA and the Federation of European Academies of Medicine ([FEAM](#)) and the European Federation for Pharmaceutical Sciences ([EUFEPS](#)). Both organisations expressed unreserved support to the guiding principles of the framework and identified education and training as a major area where fruitful collaboration can be envisaged. On this topic, close collaboration with the NCAs and the [EU-Network Training Centre \(EU-NTC\)](#) is envisaged and work is presently ongoing.

“
FEAM and its Member Academies seek to improve the health and safety of European citizens whilst promoting a creative and sustainable environment for medical research and training. As the discovery, development and regulatory oversight of novel medicines becomes increasingly complex, FEAM welcomes the publication by EMA of its new framework for collaboration with the academic community. An effective framework that facilitates communication and knowledge exchange between the regulators and expert academic groups will stimulate innovation in the development of new therapeutic approaches leading ultimately to improved patient benefit.

” **Prof. Bernard Charpentier (FEAM)**

The Agency has also actively participated in specific events with wide academic audiences - notably [“How European Biological and Medical Sciences Research Infrastructures boost Innovation by Open Access”](#) (CORBEL project, Brussels, 20 June 2017), [“Enhancing Predictivity in Medicines Development”](#) (EATRIS-ERIC Translational Medicine Conference, Prague, 24-26 September 2017), and [“Info Day, Horizon 2020 – ‘Health, demographic change and wellbeing”](#) (European Commission, Brussels, 8 December 2017) - promoting the framework and the regulatory tools presently available to support innovation in medicine development.

Overall, during the preparatory phase several activities have been set into motion, with the guiding principle of strengthening the basis from which the full framework implementation will spring successfully in the coming years.

“
We are very excited about the new framework, as it marks an important step in Europe’s continuing efforts to boost innovation at the leading edge of science. EATRIS-ERIC and our fellow European Strategy Forum on Research Infrastructures (ESFRI) look forward to contributing to the actions arising from this initiative.

” **Dr Giovanni Migliaccio (EATRIS-ERIC)**



5. Conclusions

Despite the challenging environment that EMA is currently operating in as we prepare for relocation and the operational changes resulting from Brexit, implementation of BCP has enabled an effective level of stakeholder engagement to be maintained in 2017.

In the context of engaging with stakeholders, the public hearing held in 2017 was a key event that brought all players together with their different perspectives on the same issue. The Agency will analyse the feedback and lessons learnt from this first public hearing to inform future public hearings.

While the numbers of interactions with patients and healthcare professionals continued to rise, the quality of the collaboration with these stakeholders remains an important objective. 2017 saw the green light for working with young people and work continues to bring the voice of the general practitioner to the regulatory world.

This report not only details the numbers but illustrates how valuable this input is for scientific evaluations, workshops, consultations and surveys.

Importantly, the framework for collaboration with academia has meant that the needs of this important group as well as the knowledge and expertise they can offer will be more seamlessly taken into account in the future. Work to reinforce interaction with academia has started and will continue to progress albeit at a slower pace due to BCP. With a view to the next few years and the challenges associated with relocating the Agency, the PCWP and HCPWP have agreed work plans for 2018 and 2019 outlining the areas of priority for these working parties while aligning with the interests of EMA and Europe.

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

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

6. Next steps

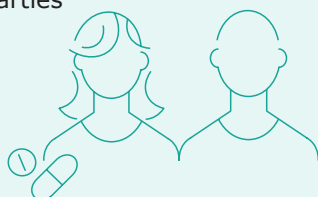
In 2018, the implications of Brexit will result in further reductions in activities involving stakeholders. The future objectives of EMA in relation to public engagement are outlined below, however they may be subject to further reprioritisation in line with BCP. A key goal will be to make best use of the limited resources available until the relocation is completed. The report for 2018 will reflect these changes.

For patients and healthcare professionals:



- ▶ Streamline eligibility renewal process for organisations
- ▶ Continued implementation of the action plan of the frameworks
- ▶ Ongoing working party topic groups
- ▶ Improve and simplify the information for the public i.e. more public-friendly and less regulatory
- ▶ Measure stakeholders' and partners' perception of EMA's engagement, information and communication activities
- ▶ Implement 2018-2019 work plans for working parties

Patients



- ▶ Further explore alternative methodologies for engagement, e.g. preference elicitation
- ▶ Implement (in part) EMA Action Plan related to recommendations on electronic Product Information
- ▶ Update action plan of framework of interaction with patients and consumers



Healthcare professionals

- ▶ Further explore ways to engage both highly specialised clinicians and primary care practitioners in regulatory activities related with innovative medicines and treatments
- ▶ Continue to facilitate early engagement of healthcare professionals in risk minimisation activities
- ▶ Initiate reflection on how to promote inclusion of young clinical researchers and practitioners in EMA activities



Academia

- ▶ Continue to interact with academia to involve them in the scientific activities of the Agency and reinforce their awareness and knowledge of the regulatory field
- ▶ Explore possible collaboration on relevant areas relating to regulatory science
- ▶ Identification of priority areas where regulatory requirements pose particular challenges for academics and their networks

Annexes

Annex I: Involvement in EMA activities (2009-2017)

Annex II: Membership of patients and healthcare professionals in EMA Management Board and Scientific Committees

Annex III: Membership of patients and healthcare professionals in EMA working parties

Annex IV: Workshops attended by patients and healthcare professionals in 2017

Annex V: Participation in EMA Workhops (2009-2017)

Annex VI: Patient and healthcare professional involvement in EMA activities (2009-2017)

Annex VII: Documents reviewed (2007-2017) by patients and healthcare professionals

Annex VIII: Eligible organisations working with EMA

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