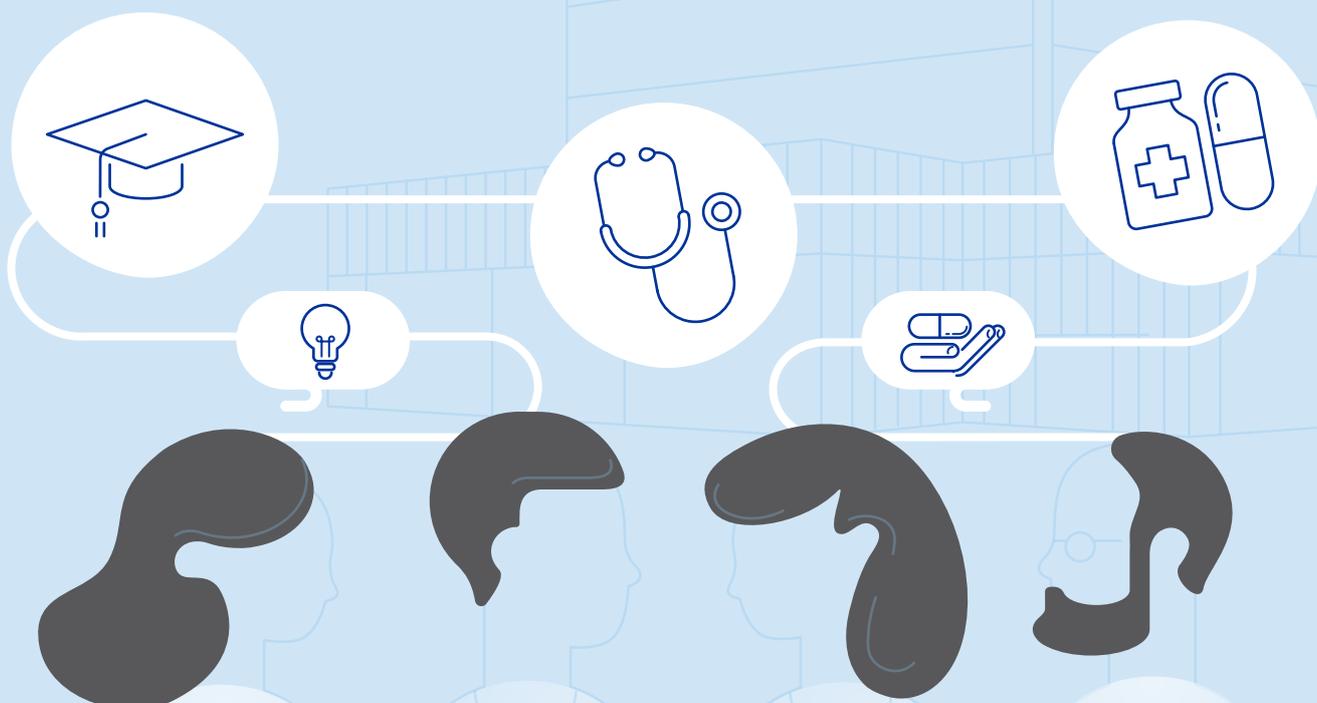




EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

STAKEHOLDER ENGAGEMENT REPORT 2018–2019

Engaging with patients, consumers,
healthcare professionals and academia



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I EXECUTIVE SUMMARY

Over the last few years EMA has faced a number of challenges, in particular the United Kingdom's withdrawal from the European Union and the Agency's subsequent relocation to Amsterdam, which was completed in mid-January 2020. In order to manage the relocation, the Agency implemented a business continuity plan (BCP) to focus on core activities essential for public health.

As part of BCP, annual stakeholder engagement reporting did not take place and a biennial report for 2018 and 2019 is presented here. During this period, stakeholder engagement in the evaluation of specific medicines remained an Agency priority while stakeholder engagement on general topics was deprioritised.

In June 2019, EMA signed a [joint statement](#) with UEMO and WONCA (two major organisations representing general practitioners) and EFPC (the main organisation representing primary care professionals in Europe) to increase collaboration with general practitioners (GPs). This will enable EMA to gain a better understanding of the impact of specific regulatory actions on patient care and to gather perspectives directly from prescribers on how medicines are used in the primary care setting. It will also raise awareness to primary care professionals on the role and activities of the EU medicines regulatory network.

Other public engagement highlights during the reporting period include:

- A second public hearing in 2018 on [quinolone- and fluoroquinolone-containing medicines](#), where the testimonies of individuals affected by these medicines brought valuable input to EMA's safety committee (PRAC) and contributed significantly to the final recommendations of the committee.
- In 2018, a multi-stakeholder [workshop](#) on electronic product information (ePI) organised jointly by EMA, Head of Medicines Agencies (HMA) and the European Commission (EC) was the result of a year-long mapping, analysis and consultation process with all stakeholders on the common [key principles](#) to guide the development and use of ePI in the EU. The next steps concern implementation of these principles and include a project that defines the common standard for ePI and provides tools for a pilot phase. Such a pilot will involve EMA and some national competent authorities to test ePI and assess the impact.
- Shortages and [availability of authorised medicines](#) remain a critical issue for patients, consumers and healthcare professionals. These stakeholders along with the pharmaceutical industry contributed to the work of the HMA-EMA taskforce on availability of authorised medicines. The taskforce was established for better prevention, identification, management and communication of availability issues. A workshop in November 2018 provided information on the taskforce's ongoing activities, its expected deliverables and reinforced the importance of stakeholders' contributions to these deliverables.
- Updates on the [Clinical Trial regulation](#) were provided during both [2018](#) and [2019](#). The purpose of the regulation is to create an environment that is favourable to conducting clinical trials in the EU with the highest standards of safety for participants and increased transparency of trial information. The Clinical Trials Information System (CTIS) will contain the centralised EU portal and database for clinical trials. EMA is collaborating with the European Commission, EU member states and ethics committees to develop the CTIS. Input from stakeholders, including patients and healthcare professionals, is critical for the development of the systems required to implement the regulation.
- Stakeholders contributed to EMA's reflection to advance regulatory science via the strategy entitled '[Regulatory Science to 2025](#)'. Responses were received from approximately 150 individuals and organisations during a public consultation and two stakeholder workshops, held in 2019, enabled further consultation and input. Final [recommendations](#) have been published.
- A multi-stakeholder meeting was held in 2019 as part of EMA's evaluation to minimise the risk of serious errors seen with use of [methotrexate](#). The discussion resulted in recommendations such as introduction of

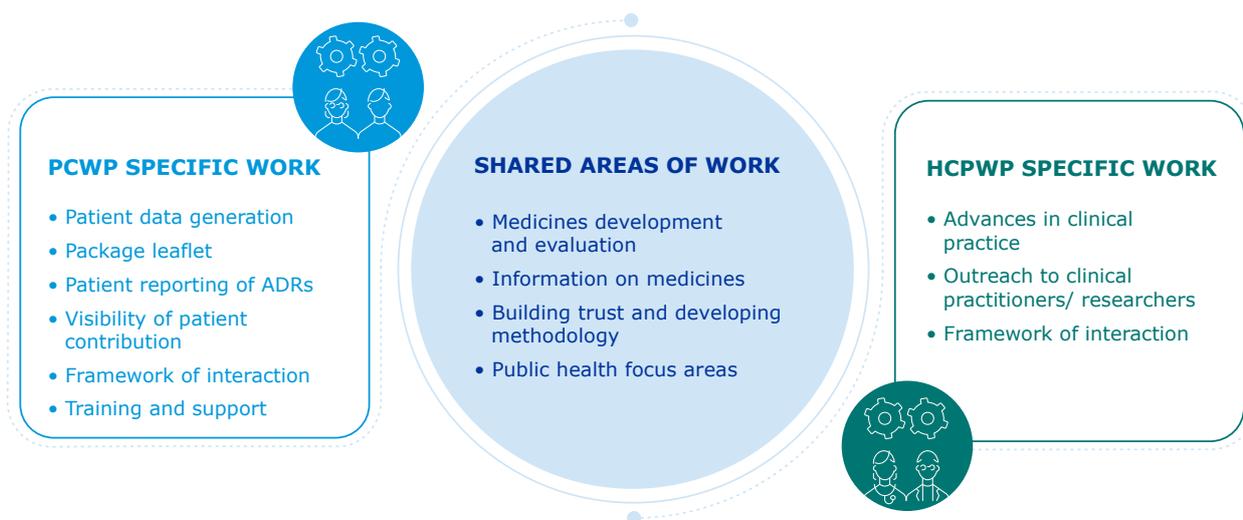


Figure 1: Focus areas for the Patients and Consumers Working Party (PCWP) and Healthcare Professionals Working Party (HCPWP) for 2019-2022.

patient cards and checklist for prescribers/ pharmacists to facilitate discussion with patients.

- As part of a lessons learnt exercise following the detection of [nitrosamine impurities](#) in sartan blood pressure medicines (also known as angiotensin II receptor blockers), a multi-stakeholder meeting was organised in 2019 with representatives from healthcare professional and patient organisations, industry associations, and international regulators. Critical input feeding into the preparation of a [public report](#) was obtained from stakeholders, including healthcare professionals' request for information to be shared earlier to be able to address concerns from their patients. Similarly, patients requested regulatory authorities to provide early advice and a clear description of the risk in lay language.
- The document that describes the [role of EMA committee members representing patients and healthcare professionals](#) was updated to include information on activities supporting these members' participation in the different EMA scientific committees as well as information on the European Commissions' nomination process.
- An informative booklet describing the journey of an authorised medicine '[From lab to patient](#)' was developed and is available in several EU languages. It describes how a

medicine authorised via EMA is developed and progresses through the regulatory path into evaluation until approval and marketing.

- Current methodologies for patient engagement during benefit-risk evaluation by CHMP have been expanded with emphasis on written consultations in addition to ongoing oral explanations and expert participation in scientific meetings.

Despite the limitations of BCP, the Patients and Consumers Working Party (PCWP) and Healthcare Professionals Working Party (HCPWP) still managed to hold virtual and face-to-face meetings, which enabled continued exchange on important topics, including the preparation of the new working party mandates.

A [three-year joint workplan](#) (2019-2022) for PCWP and HCPWP has been developed. The new mandates began in June 2019 for both working parties and co-chairs were elected during the September meetings. Main areas included in the shared workplan are shown in Figure 1.

FUTURE STEPS



The focus of future activities will be enhanced collaboration.

The Agency together with the network of European medicines' agencies has been working to set up its strategic priorities for the next five years, shaping them in close cooperation with all stakeholders. While much has already been done, efforts should aim for earlier and more meaningful engagement with stakeholders.

Two strategic documents developed in consultation with stakeholders have been published, which will mark the future direction of public and stakeholder engagement activities.

- The [Regulatory Science Strategy to 2025](#) was published after a public consultation and workshops with stakeholders. Five key goals were identified in the strategy. Patient and healthcare professional engagement will be particularly critical in the implementation of the goals a) to drive collaborative evidence generation to improve the scientific quality of evaluations and b) to advance patient-centred access to medicines in partnership with healthcare systems.
- EMA and the Heads of Medicines Agencies also developed the joint European medicines agencies network (EMAN) [strategy](#) to 2025. Six priority areas have been described that include: availability and accessibility of medicines, data analytics, digital tools and digital transformation, innovation, antimicrobial resistance and other emerging health threats, supply chain challenges, sustainability of the network and operational excellence.

Implementation and progress in many of the highlighted areas will require input from the prescribers and end users. The voices and perspectives of patients, healthcare professionals and academia are essential for the success of the strategies.

Another important priority stemming from the current COVID-19 pandemic will be to review the lessons learned to rapidly and effectively address health threats in the future.

Shortages of medicines remain a priority and the [HMA/EMA task force](#) is drafting a concept paper providing patient and healthcare professional organisations with principles and examples of

good practices for shortage prevention and management. A multi-stakeholder workshop is planned to follow-up on developments brought forward by the EC Pharmaceutical Strategy and the EMAN strategy to 2025.

Looking ahead, EMA is committed to ensuring that the patient voice is systematically incorporated throughout medicine development and evaluation, including during evidence generation and will:

- explore additional methodologies to gather and use patient experience data from the wider patient community;
- develop guidance on the collection and use of patient experience data;
- enhance patient involvement in EMA scientific committees through various methodologies such as increased written consultations and preference elicitation;
- support global alignment on the scientific methodology to gather patient contribution to medicines development.

EMA is also enhancing interactions with healthcare professionals, including doctors, pharmacists and nurses, to:

- access the best possible independent expertise in clinical practice, to incorporate the real-world experience of the full spectrum of healthcare (from primary, to secondary and tertiary care) into medicine development, benefit/risk evaluation and monitoring;
- contribute to a more efficient and targeted communication to healthcare professionals, supporting the translation of risk minimisation measures into clinical guidelines and implementation into routine clinical practice;
- better manage and prevent shortages of authorised medicines;
- explore opportunities for engaging with healthcare professionals in clinical research and practice on areas such as clinical trial design, personalised medicine, and use of real-world evidence;
- enhance healthcare professionals' organisations' understanding of the role and activities of the EU medicines regulatory network

Future interactions and engagement with academia and innovators will be co-ordinated by the Regulatory Science and Innovation Task Force within EMA

- EMA will continue to collaborate with and provide support to several EU-wide research and development platforms and consortia to explore synergies and foster development of curative and personalised treatments (e.g. Horizon 2020, Innovative Medicines Initiative (IMI), European Joint Programme on Rare Diseases (EJPRD));
- EMA will increase outreach to and reinforce collaboration with the academic community to address rapidly emerging research questions and to foster research in strategic areas of regulatory science;
- EMA aims to further engage with and train (early career) researchers in regulatory science (e.g. EMA traineeship and Collaborating Expert programmes and support to CSA-STARS project).

Training of stakeholders on the role and activities of EMA and where they can be involved is an important activity. Building on experience so far, the existing training strategy for patients will be revised, and will also include training for healthcare professionals.

We take this opportunity to thank our stakeholders for all their hard work and support and look forward to continued collaboration and an exploration of new methodologies for engagement in Agency activities.

This Stakeholder report on interactions with patients, healthcare professionals and academia was presented to the Management Board during its December meeting, 2020. EMA's interactions with Industry stakeholders are presented in a separate report.

| 2018 HIGHLIGHTS

EUROPEAN REFERENCE NETWORKS (ERN)

This [workshop](#) explored how EMA and ERNs can reinforce each other's efforts to encourage and facilitate research into new treatments for rare and low-prevalence diseases and how ERNs could be engaged in EMA activities.

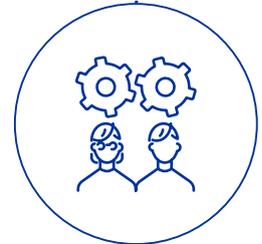


AVAILABILITY OF AUTHORISED MEDICINES

A [multi-stakeholder workshop](#) united patients, consumers, healthcare professionals, regulators and pharmaceutical industry to discuss the availability of medicines and how to contribute to the deliverables of the Heads of Medicines Agencies/EMA task force. The task force aims to coordinate actions for better prevention, identification, management and communication of availability issues.

REGULATORY AWARENESS SESSION

An [awareness session](#) aimed at academics, NGOs staff and regulators from and outside the European Union was organised. This workshop provided insight into the functioning of the EU regulatory network, the role of EMA, the work of EMA and interactions with scientific experts and stakeholders.



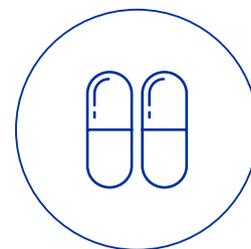
EU-INNOVATION NETWORK

This [workshop](#) addressed challenges and opportunities for regulatory science in the medicines research space to boost the success of development programmes and clinical studies. The discussion included national and EU initiatives and creating synergies to support new medicines and innovative healthcare solutions.

| 2019 HIGHLIGHTS

METHOTREXATE CONTAINING MEDICINAL PRODUCTS – STAKEHOLDER MEETING

A meeting with patients representing rheumatoid arthritis, psoriasis and Crohn’s disease groups, healthcare professional specialists and medication error experts was held to discuss new measures to prevent serious and potentially fatal errors with the [dosing of methotrexate](#).



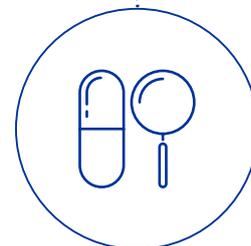
STRENGTHENING ENGAGEMENT BETWEEN EMA AND GENERAL PRACTITIONERS

EMA and the European Union of General Practitioners (UEMO), the European section of the World Organization of Family Doctors (WONCA) and the European Forum for Primary Care (EFPC), signed a [joint statement](#) committing to strengthening interaction between EMA and this important group of healthcare professionals.



MEETING ON LESSONS LEARNT FROM SARTAN MEDICINES CONTAINING NITROSAMINE IMPURITIES

A multi-stakeholder meeting was held discuss how to better prevent and manage cases of [sartan medicines](#) containing nitrosamine impurities. The meeting brought together patients and healthcare professional organisations, industry associations and EU and international regulatory authorities.



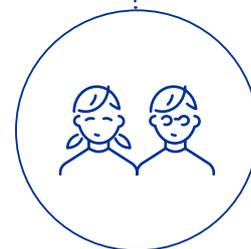
REPORT OF HEALTHCARE WORKERS’ KNOWLEDGE ON RESPONSIBLE USE OF ANTIBIOTICS

A multi-country and multi-country and multi-professional study on the knowledge, attitudes and behaviours of healthcare workers regarding [antibiotics](#), [antibiotic use and antibiotic resistance](#) across 30 EU and European Economic Area (EEA) countries was published by the European Centre for Disease Control (ECDC). Results were presented to the PCWP and HCPWP and members of the HCPWP contributed to the survey.



PROGRESS ON INVOLVING YOUNG PEOPLE IN EMA ACTIVITIES

In the context of [engaging with young people](#), a representative from the European Patient Forum (EPF) youth group addressed the Paediatric Committee (PDCO) and presented the group, their goals, projects and training. Collaboration with the Enpr-EMA network is being explored. The PDCO continues to use surveys to reach out to young people in the context of evaluation of the paediatric investigation plans for development of medicines for paediatric indications.



In developing this biennial report, the format and structure has been updated and streamlined taking into account experience with [previous reports](#). This report is organised into sections that describe activities with patients, healthcare professionals (HCP) and academia.

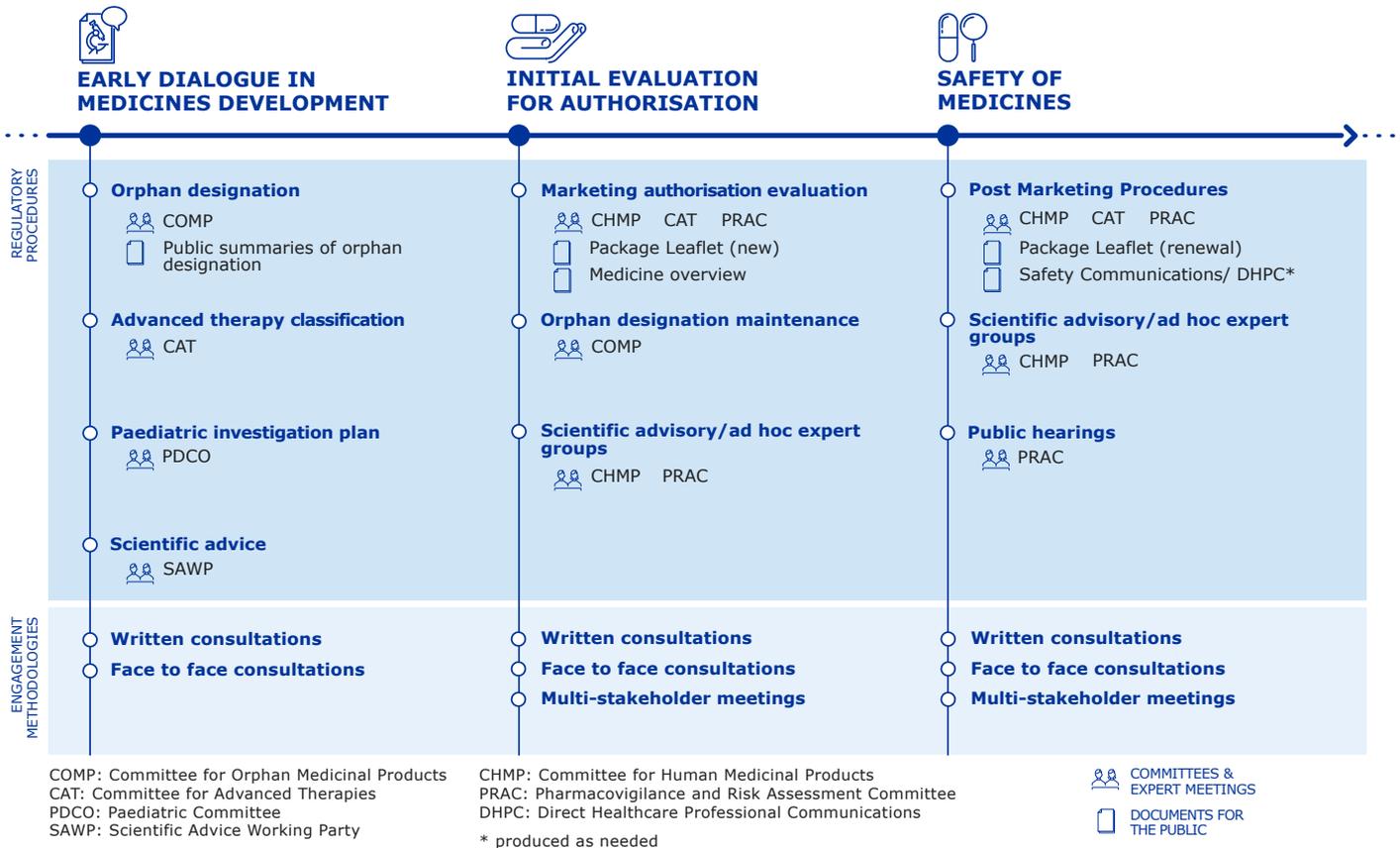


Figure 2: Patient and healthcare professional involvement in EMA activities all along the regulatory lifecycle.

Each section is further organised by phase of the medicine regulatory lifecycle (Figure 2), demonstrating the various activities, input and added value of stakeholder contribution along the medicine's journey through EMA.

INVOLVEMENT OF PATIENTS



[EARLY DIALOGUE IN MEDICINES DEVELOPMENT](#)

[INITIAL EVALUATION FOR AUTHORISATION](#)

[SAFETY OF MEDICINES](#)

INVOLVEMENT OF HEALTHCARE PROFESSIONALS



[EARLY DIALOGUE IN MEDICINES DEVELOPMENT](#)

[INITIAL EVALUATION FOR AUTHORISATION](#)

[SAFETY OF MEDICINES](#)

ACTIVITIES WITH ACADEMIA



[TARGETED ENGAGEMENT](#)

[EXTERNALLY FUNDED PROJECTS](#)

[INNOVATION TASK FORCE](#)

More efficient methodology for quantitative reporting has been introduced with the focus being put on the number of medicine-related activities where patients and healthcare professionals were involved. This decision was made during BCP in consultation with the PCWP, HCPWP and eligible organisations combined with internal discussions.

The number of medicine-related activities compared to non-medicine related activities where patients (Figure 3) or healthcare professionals (Figure 4) have been involved are shown per year. The activities represented by these numbers are diverse and span the whole medicines regulatory life cycle.

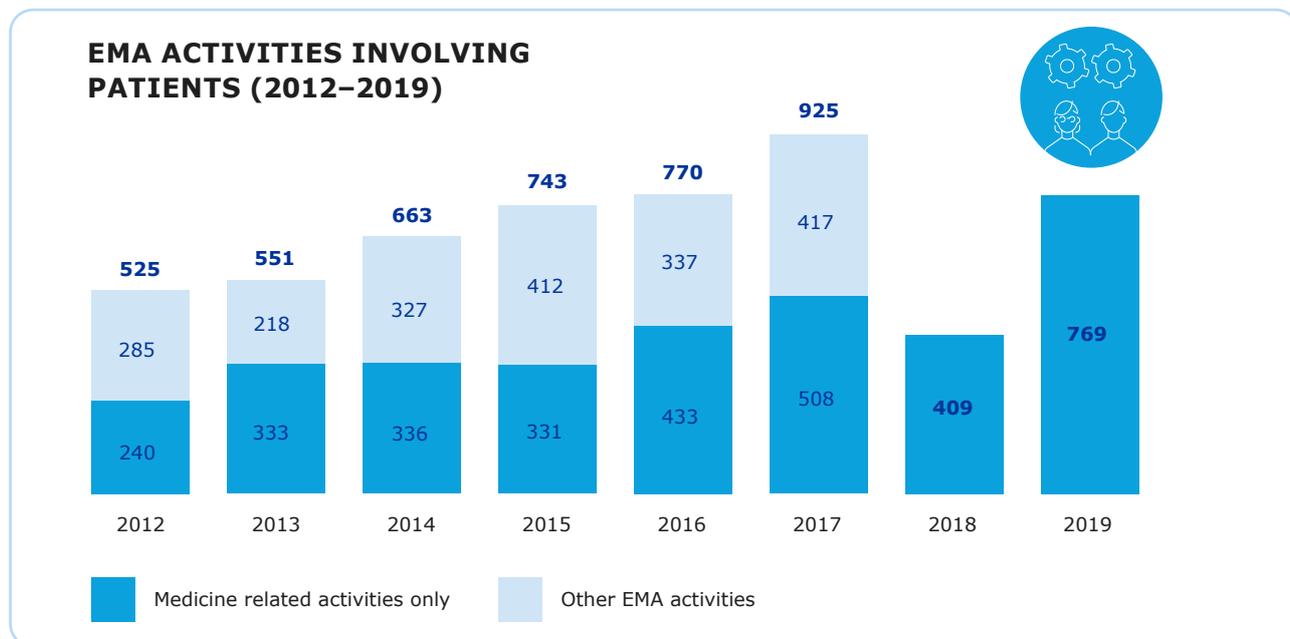


Figure 3: Numbers of non-medicine related activities involving patients (2012 – 2019) are shown in pale blue and medicine-specific activities in dark blue. The number of medicine-related activities in 2019 almost reached the total of activities of 2017*. This sharp increase is due to the high number of responses received with the surveys conducted in the context of written consultations from EMA committees.

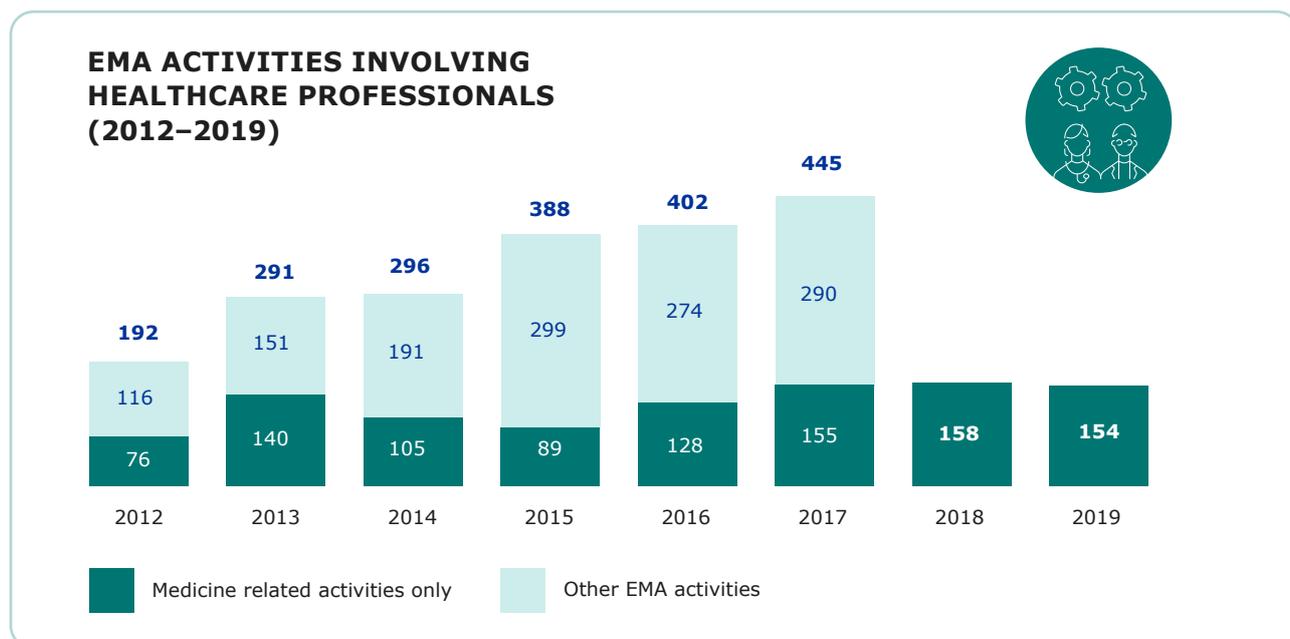


Figure 4: Numbers of non-medicine related activities involving healthcare professionals are shown in pale green and medicine-related activities in green. As can be seen in green, the number of medicine-specific activities involving healthcare professionals in 2019 has remained constant from 2017*.

* Other EMA activities were not reported in 2018–19 due to BCP

PATIENTS' AND CONSUMERS' AND HEALTHCARE PROFESSIONALS' WORKING PARTIES

The Patients' and Consumers' (PCWP) and Healthcare Professionals' (HCPWP) working parties held the following meetings in 2018 and 2019:



2018

- [April](#) meeting focused on digital media and health including real world evidence, mhealth and social media
- [September PCWP](#) meeting highlighted patient involvement and visibility in EMA activities
- [September HCPWP](#) meeting included a reflection on advances in clinical practice and regulatory challenges
- [September PCWP/HCPWP](#) joint meeting included Regulatory Science to 2025, pharmacovigilance in pregnancy, ePI and access to medicines
- December was the first virtual meeting due to EMA's relocation



2019

- May virtual meeting – focus on medicine shortages
- June virtual meeting – end of previous mandate and description of three-year workplan
- [September PCWP](#) – co-chair election for PCWP, Regulatory Science to 2025, CIOMS guidance of patient involvement in medicines development
- [September HCPWP](#) – co-chair elections for HCPWP, Regulatory Science to 2025, Knowledge transfer
- [September PCWP/HCPWP](#) joint meeting included vaccines, impact of pharmacovigilance and repurposing activities
- [November](#) meeting with all eligible organisations highlighted new legislations and EMA in 2020

The PCWP/HCPWP mandates were renewed (2019-2022) and [highlights](#) of the previous mandate were published. For the first time a [three-year workplan](#) was proposed and adopted.

With the completion of the previous mandate there was a call for new members and nominations to the working parties. Membership of the working parties ([PCWP/HCPWP](#)) was expanded from 20 to 22 members following an increase in the number of EMA eligible organisations for both [patients](#) and [healthcare professionals](#).

In line with the new mandates, the working parties elected their co-chairs, during the September meetings. Juan Garcia Burgos remains as EMA co-chair of both working parties.



Kaisa Immonen (EPF) was re-elected as co-chair of the PCWP



Ulrike Jaeger (EHA) was elected as new co-chair of the HCPWP

ACTIVITIES INVOLVING INDIVIDUAL PATIENT EXPERTS

Early dialogue with patients

Patient engagement is critical at all stages of medicines development with early dialogue playing a particularly important role. At EMA, patients are involved as full voting members of the COMP, CAT and PDCO. In addition, patient experts can be consulted by committees on specific issues related to medicines either in person or in writing. Patients are actively involved in scientific advice procedures where they contribute to clinical trial design and provide input on what is clinically meaningful.

CONSULTATIONS BY EMA SCIENTIFIC COMMITTEES



During the review of a medicine, EMA scientific committees may need to reach out to patients with experience of the condition being treated in order to obtain specific information on issues such as

those described below.

The **Paediatric Committee (PDCO)** consulted patients and carers using surveys during the review of paediatric investigation plans (PIPs) on two occasions in 2018 receiving a total of 43 responses and three occasions in 2019 with a total of 200 responses, both surveys contributed to the assessment of the proposed plans.

The aim of the surveys was to gather the views of patients on the burden of their condition, its current treatment, the risks they would accept for potential benefit as well as the definition of improvements that would be meaningful.



EARLY DIALOGUE IN MEDICINES DEVELOPMENT



The **Committee for Orphan Medicinal Products (COMP)** consulted patients during the evaluation of the criteria for orphan designation for five medicines in the therapeutic areas of oncology, neurology, haematology and respiratory. They were all invited to plenary meetings of the committee with the companies.

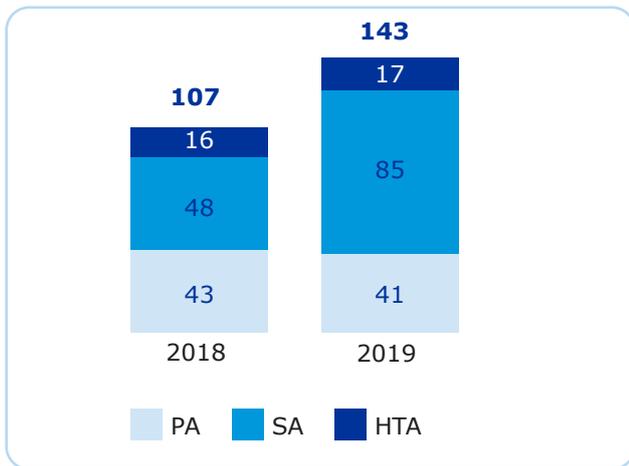
Patients were consulted by the **Committee for Advanced Therapies (CAT)** during a discussion on gene therapy in haemophilia.

PAEDIATRIC COMMITTEE (PDCO) SURVEYS



2018
43
responses

2019
200
responses



SCIENTIFIC ADVICE PROCEDURES



A total of 107 patients in 2018 and 143 patients in 2019 were involved in scientific advice (SA), protocol assistance (PA) and parallel advice with health

bodies. The chart shows the breakdown of their involvement by procedure type for both years.

EMA AND HEALTH TECHNOLOGY ASSESSMENT BODIES



EMA and the European network for health technology assessment (EUnetHTA) published a three-year [work plan](#) (2017-2021) that outlines several areas of focus for collaboration. One of these areas

is patient and healthcare professional engagement.

EMA and EUnetHTA participate in biannual meetings and contribute to stakeholder meetings and exchange regularly on best practices for

stakeholder engagement on parallel scientific advice and joint assessment procedures. EMA was consulted for the identification of patients and healthcare professionals for two joint assessments in 2018 and for six joint assessments in 2019.

When medicines developers request parallel scientific advice at EMA, patients are systematically invited to participate in meetings involving the regulators and HTA bodies.

SCIENTIFIC ADVICE - WHERE PATIENTS GAVE INPUT?



Added value of patient input in scientific advice:



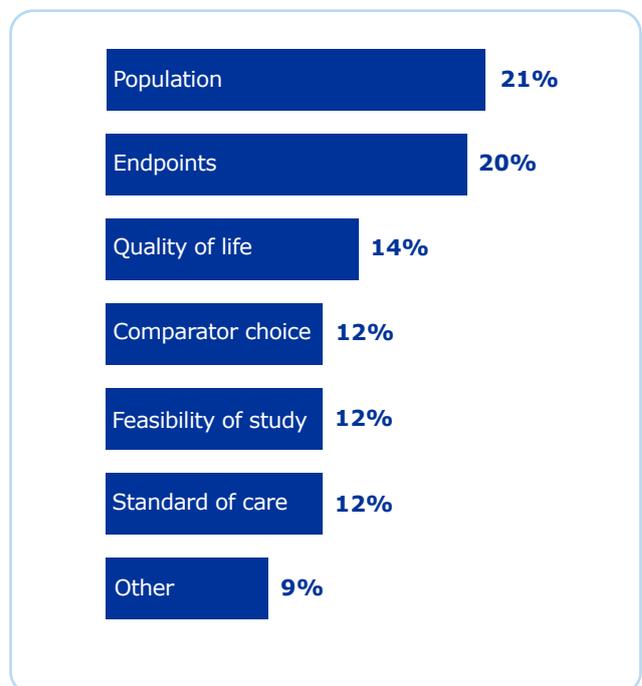
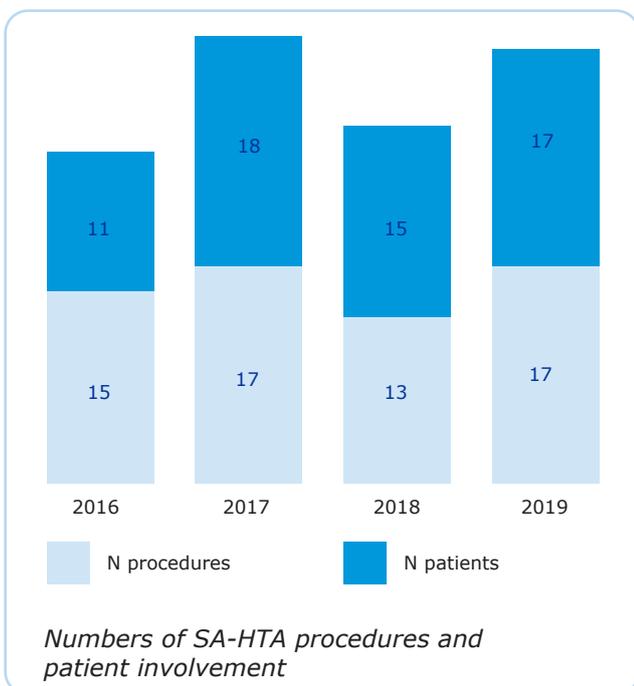
Colleagues responsible for each scientific advice procedure were surveyed about the aspect of

the medicines development plan where patients provided most input.

Responses indicated that patients provided valuable input on the population selected for the trial, endpoints, comparators and quality of life to name a few.

'Other' contributions from patients included perspectives on the study duration, dosage and study relevance.

Consistently, advice provided to companies by the Scientific Advice Working Party was changed based on input from patients in 25% of cases and in the majority of cases (90%) patients agreed with the proposed development plan.



Patient involvement in initial evaluation for authorisation

Evaluation of medicines for marketing authorisation is primarily the responsibility of the Committee for Human Medicinal Products (CHMP). While the committee membership does not include patients, when needed the CHMP invites patients to attend meetings in person and also consults patients in writing.

COMMITTEE CONSULTATIONS



During the evaluation of a medicine, the pharmaceutical company may be invited to attend the CHMP meeting to respond to questions raised by the committee. Depending on the specific issues raised, a patient may also be invited to give their perspective on the issues under discussion. It is very valuable for the committee to hear directly from those living with the condition who will be directly affected by the regulatory decision.

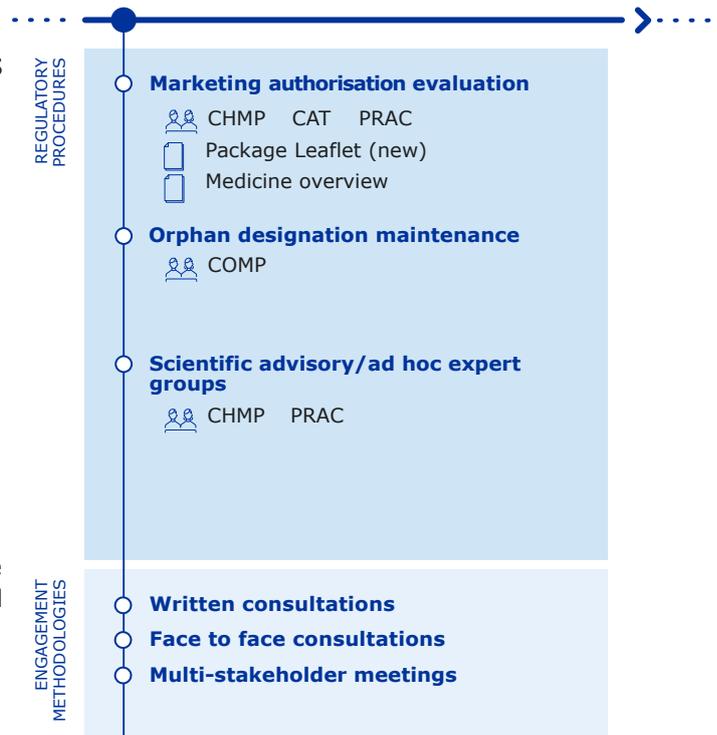
In 2018, the CHMP consulted nine patients during the evaluation of five medicines and in 2019, ten patients attended six CHMP meetings to provide input and share their opinions on the benefits and the risks related to the particular medicine under discussion.

Different methodologies are in use for consulting patients and in 2019 for one evaluation the CHMP also consulted patients in writing on questions related to the burden of the disease, current treatments, quality of life and what risks they would accept for potential benefit of a medicine. They received 52 responses and the information received was taken into consideration during the evaluation.

Review of orphan status of a medicine by the **Committee for Orphan Medicinal Products (COMP)** prior to marketing authorisation. The COMP consulted patients while reviewing whether a medicine that received a positive opinion from the CHMP still fulfilled the criteria for orphan



INITIAL EVALUATION FOR AUTHORISATION



designation. The questions focused on the significant benefit of the medicine, in particular its preparation and administration, and COMP received 24 responses from patients.

A patient expert was invited to the COMP meeting to present the results of the survey and to complement with their own personal experience. The survey helped the committee to better understand the situation in the different EU member states and reach a decision.

SCIENTIFIC ADVISORY (SAG) AND AD HOC EXPERT GROUPS



SAG and ad hoc expert group meetings are convened by the CHMP, or the PRAC, to provide advice in connection with ongoing assessments of specific medicines or treatments. These

meetings involve in-depth discussions with experts (including patients) on outstanding issues that need to be resolved prior to the committee being able to conclude on the safety and efficacy of the medicine.

In 2018, 37 patients/carers participated in 26 SAG/ad hoc expert meetings and in 2019, 46 patients/carers were involved in 25 of these consultations.

The therapeutic areas covered by these meetings include oncology, neurology, viral diseases, anti-infectives and cardiovascular diseases.

Patients receive the same information as all other experts in the meetings and contribute by providing valuable information in relation to the efficacy of the medicine and the safety profile that is acceptable to them.

REVIEW OF EMA DOCUMENTS ADDRESSED TO PATIENTS AND THE GENERAL PUBLIC



Documents destined for patients and the general public are reviewed by patients prior to publication.

The suggestions and comments made by patients help to ensure that the documents address the targeted audience and use the right language to ensure the message is clear and understandable.

Patient input is very impactful with an average of 50% of comments incorporated into the final versions. In addition, changes to the document templates have been made following patients' suggestions.



282

**REVIEW
OF EMA
DOCUMENTS**

2018

42

**MEDICINE
OVERVIEWS**

74

**PACKAGE
LEAFLETS**

16

**HERBAL
SUMMARIES**

2019

38

**MEDICINE
OVERVIEWS**

101

**PACKAGE
LEAFLETS**

11

**HERBAL
SUMMARIES**

Patient involvement in safety activities

Once a medicine is authorised and available on the market, there is continued monitoring of its efficacy and safety. The committee involved in these activities is the Pharmacovigilance Risk Assessment Committee (PRAC). In addition to the two patients who are members of this committee, many other patients are consulted for additional safety related activities including multi-stakeholder meetings on issues arising such as adverse side effects, medication errors and manufacturing issues. Some safety activities can also occur prior to authorisation, for example the development of risk minimisation measures, or requirements for follow up studies.

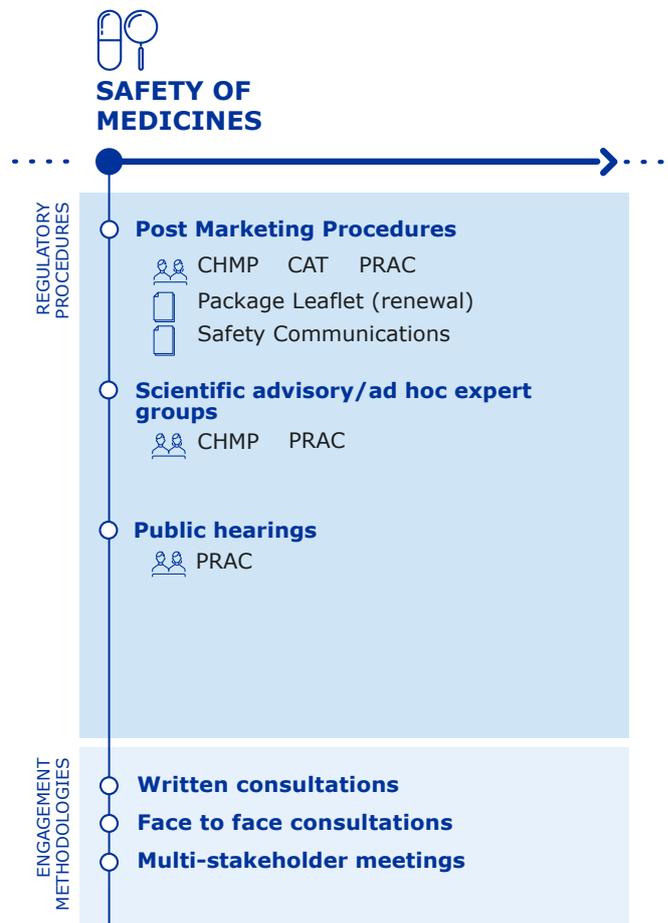
MULTI-STAKEHOLDER MEETINGS



Methotrexate containing medicines

A meeting with patients representing rheumatoid arthritis, psoriasis and Crohn's disease groups, healthcare professional specialists and medication error experts was held to discuss new measures to prevent serious and potentially fatal errors with the dosing of methotrexate. The group discussed issues around packaging, prescription process, dosing schedule as well as what would be the most appropriate tools and measures to raise awareness.

The PRAC examined the available evidence and recommended additional measures to reduce dosing errors so that benefits of methotrexate-containing medicines continue to outweigh their risks. The measures were agreed after consultation with patients and healthcare professionals.



Nitrosamine impurities in sartan medicines

A multi-stakeholder meeting on [nitrosamine impurities](#) found in several medicines known as 'sartans' was organised with healthcare professional and patient organisations, industry stakeholders, and international regulators. Critical input was obtained from stakeholders with healthcare professionals requesting that information be shared early enough for them to be able to address concerns from their patients. Similarly, patients requested regulatory authorities to provide advice and a clear description of the risk in lay language. A survey was conducted asking stakeholders about their awareness of the impurities and subsequent actions and communications related to the issue. The data gathered contributed to the [lessons learnt](#).

CONSULTATIONS BY SAFETY COMMITTEE



Patients were consulted by the PRAC during a safety review of rituximab in relation to new educational materials that would be used to raise awareness of specific risks. Where necessary,

educational materials can be included with a medicine in addition to the package leaflet.

A survey was conducted with patients to determine which of the materials proposed they felt best addressed their needs. A total of 61 responses were received. The responses assisted the committee to determine the optimal materials to best inform patients of the potential side effects of the medicine under review.

RELEVANT DOCUMENT REVIEW – SAFETY COMMUNICATIONS



Safety communications are used to convey important information about the risks of a medicine, for example if a new serious side effect is observed or when a known side effect is more

serious than previously thought. In some cases, a safety communication is published to announce the start of a review of a new risk or at the end of the review when advice for patients and healthcare professionals is provided.

In 2018, 19 safety communications were reviewed by 35 patients and in 2019, 10 safety communications were reviewed by 11 patients which helped ensure that the messages were clear and complete for the target audience.



101

**SAFETY
COMMUNICATIONS
REVIEWED BY
PATIENTS**

2018

19

2019

10

ACTIVITIES INVOLVING INDIVIDUAL HEALTHCARE PROFESSIONAL EXPERTS

Healthcare professional involvement in medicines development

In this early stage of medicines development, healthcare professionals are involved as members of committees and can also be consulted by committees on specific issues related to medicines. Consultation of practicing clinicians can provide valuable insight into unmet needs, current medicines in use and how they are used as well as a perspective based on real life of how regulatory decisions can be translated into clinical practice.

INVOLVEMENT IN EARLY DIALOGUE



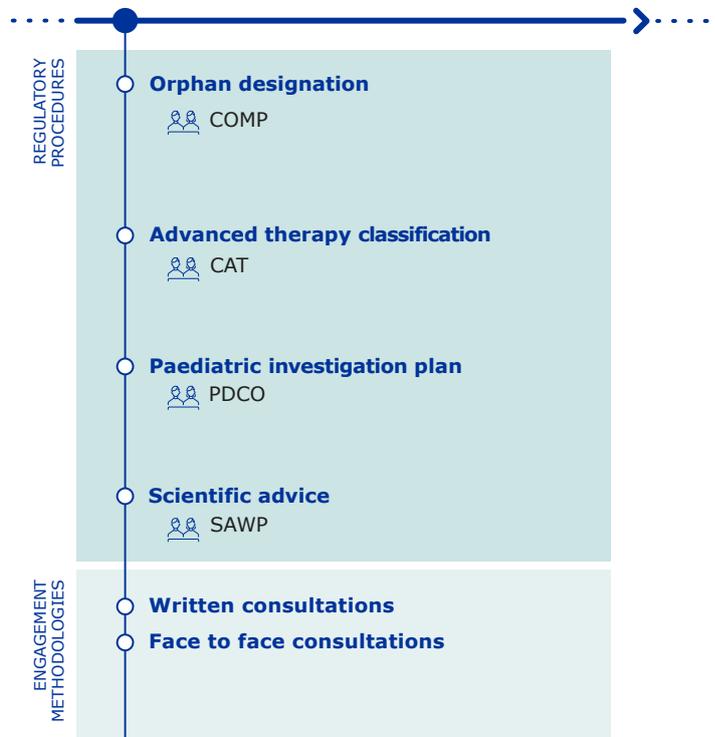
EMA works with a number of healthcare professional organisations and learned societies. They are consulted on many diverse aspects related to medicines.

They can contribute extensively to advice to companies during the development of medicines. The perspectives of practicing clinicians, specialised nurses and pharmacists are critical to discussions around the real-life implications of clinical study design and clinical benefit of candidate medicines.

EMA is developing methodology of healthcare professional engagement with the Scientific Advice Working Party to reinforce and expand knowledge and experience already existing within the regulatory network.



EARLY DIALOGUE IN MEDICINES DEVELOPMENT



CONSULTATIONS BY EMA SCIENTIFIC COMMITTEES



Healthcare professionals were consulted by the **Committee for Advanced Therapies (CAT)** during a discussion on gene therapy in haemophilia.

Healthcare professionals' in initial evaluation for authorisation

Evaluation of medicines for marketing authorisation is primarily the responsibility of the Committee for Human Medicinal Products (CHMP). While there are no members representing EU healthcare professionals as with other committees, they are consulted by the committee on diverse aspects during the evaluation of the medicine for marketing authorisation.

SCIENTIFIC ADVISORY (SAG) AND AD HOC EXPERT GROUPS



SAG and ad hoc expert groups are meetings that are convened by the CHMP, or the PRAC, to provide advice in connection with ongoing assessment of specific medicines or treatments. These

are in-depth discussion meetings with experts (including additional healthcare professionals) on outstanding issues that need to be resolved prior to the committee being able to conclude on the safety and efficacy of the medicine.

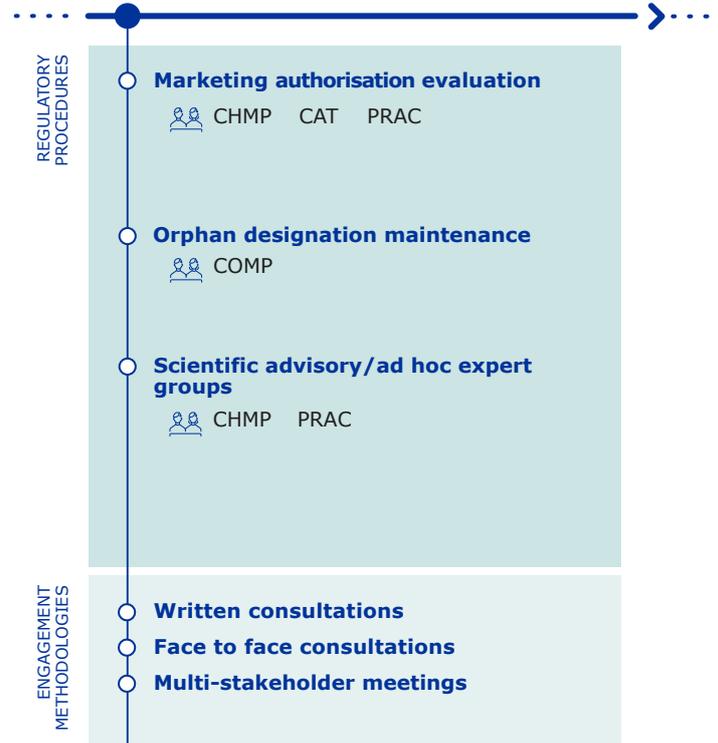
In 2018, 31 healthcare professionals took part in 18 SAG/ad hoc expert meetings and in 2019, 36 healthcare professionals were involved in 15 SAG/ad hoc expert consultations. The therapeutic areas covered by the scientific advisory or ad hoc expert group meetings include oncology, neurology, viral diseases, and cardiovascular diseases.

Some of the outstanding issues that were addressed where specialist healthcare professionals contributed include the current clinical practice in use, population that was treated, the clinical relevance of the effects measured by the study, effects on disease progression and the position of the medicine under evaluation amongst the other treatment options to name a few.

Several of the experts invited to participate in SAG/ad hoc expert meetings were identified via the [European Reference Networks](#).



INITIAL EVALUATION FOR AUTHORISATION



CONSULTATION OF HEALTHCARE PROFESSIONALS BY EMA SCIENTIFIC COMMITTEES



Following a positive opinion by the CHMP, a medicine was reviewed by the **Committee for Orphan Medicinal Products (COMP)** to determine whether it still fulfilled the criteria for orphan designation.

The committee consulted specialist healthcare professionals such as haematologists, hospital pharmacists and oncology pharmacists in this review. The questions focused on the preparation and administration of the existing treatment and received 44 responses from healthcare professionals in 24 European countries. This valuable input from healthcare professionals helped in particular to inform the COMP of the current situation and practice in Europe and contributed to their deliberation during the assessment of the maintenance of the orphan designation for this medicine.

Healthcare professional involvement in safety of medicines

Once a medicine is authorised and available on the market, monitoring of its safety continues. The committee involved in these activities is the Pharmacovigilance Risk Assessment Committee (PRAC) and in addition to the two healthcare professionals who are members of this committee, many others are consulted for additional risk minimisation activities.

COMMITTEE CONSULTATIONS



In 2018, healthcare professionals specialised in various fields were consulted by the safety committee regarding various issues including medication errors, wording on product information and patient information leaflets.

In 2019, following the PRAC recommendation to restrict use of **fluoroquinolone and quinolone antibiotics**, a dialogue was initiated with the European Association of Urology (EAU) to make it clear that fluoroquinolones should be reserved for treating bacterial prostatitis and must not be used inappropriately when treating chronic prostatitis (CP) and chronic pelvic pain syndrome (CPPS). As a result, EAU recommended that guidelines on chronic prostatitis should be revised accordingly and lent its support to communicating through publications and their annual conference to promote a change in practice. This will ultimately lead to a more rational use of antibiotics and will limit the unnecessary exposure of patients to potentially persistent and seriously disabling side effects.

MULTI-STAKEHOLDER MEETINGS

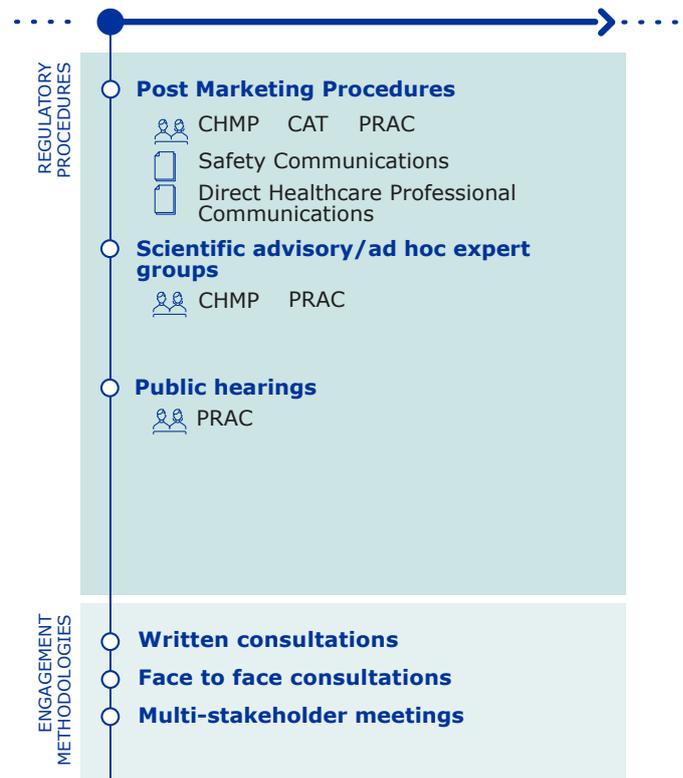


Methotrexate containing medicines

A meeting with patients representing rheumatoid arthritis, psoriasis and Crohn's disease groups, healthcare professional specialists and



SAFETY OF MEDICINES



medication error experts was held to discuss new measures to prevent serious and potentially fatal errors with the dosing of methotrexate.

The PRAC examined the available evidence and recommended additional measures to reduce dosing errors so that benefits of methotrexate-containing medicines continue to outweigh their risks. The measures were agreed after consultation with patients and healthcare professionals.

Nitrosamine impurities in sartan medicines

A multi-stakeholder meeting on [nitrosamine impurities](#) found in several medicines known as 'sartans' was organised with healthcare professional and patient organisations, industry stakeholders, and international regulators. Critical input was obtained from stakeholders with healthcare professionals requesting that information be shared early enough for them to be able to address concerns from their patients.

General practitioners and community pharmacists were particularly impacted by this situation as they are the frontline in prescribing and dispensing of medicines. They had to explain the situation to patients and find alternative treatments. A survey was conducted asking stakeholders about their awareness of the impurities and subsequent actions and communications related to the issue. The data gathered contributed to the [lessons learnt](#).

DOCUMENT REVIEW – SAFETY COMMUNICATIONS



The PRAC and CHMP published 22 communications in 2018 and 16 communications in 2019 on issues related to the safety of medicines and prior to publication, these were reviewed

by a total of 75 healthcare professionals.

Safety communications are used to convey important information about the risks of a medicine. The review of these documents by healthcare professionals is beneficial for ensuring that the advice provided is relevant and implementable in practice.

[Direct healthcare professional communication \(DHPC\)](#) are sent to healthcare professionals to inform them of important new safety information about a medicine and any actions they should take.

DHPCs serve to inform healthcare professionals of a suspension, withdrawal or revocation of a marketing authorisation for safety reasons, a restriction of indication, a change in the recommended dose, a supply shortage or issues with the quality of a medicine.

In 2018, 23 DHPC were reviewed by 40 healthcare professionals and in 2019, six DHPC letters were reviewed by 13 healthcare professionals ensuring that the message is meaningful and clear for the target audience. Review of DHPCs was primarily carried out by general practitioners and input based on their daily practice is of great value.



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SAFETY COMMUNICATIONS REVIEWED BY HEALTHCARE PROFESSIONALS

2018

22

2019

16



29

DHPCs REVIEWED BY HEALTHCARE PROFESSIONALS

2018

23

2019

6

ACTIVITIES INVOLVING ACADEMIA

In 2018 and 2019, EMA continued to work towards the implementation of the [Framework](#) of collaboration with academia focusing on priority areas where regulatory requirements pose a challenge for academia and their networks. In this light, EMA explored options for possible incentives such as [reduction in fees](#) for regulatory procedures. Other activities are described below.

COMMUNICATION ACTIVITIES



Since the implementation of the framework for collaboration with Academia, the agency has created an EMA entry point for academia to receive information on available support within the EU Regulatory Network and has create a [dedicated space for academia](#) on the Agency website.

- providing regulatory and scientific support to foster the development of new and innovative medicines.

EXTERNALLY FUNDED PROJECTS



EMA is involved in a number of research projects with academia, learned societies and research groups, as part of its mission to foster scientific excellence in the evaluation and supervision of medicines. The agency believes that ensuring research is performed to regulatory standards, helps translate the results into public health benefits.

The research projects are mostly funded by [Horizon 2020](#), the largest ever European Union (EU) funding programme for research and innovation. The Agency provides [support to medicine developers and researchers applying for funding](#) and engages in externally funded projects either as consortium members or as part of an advisory board.

The Agency also supports other Horizon 2020 initiatives, including Marie Skłodowska-Curie actions (MSCA), that provide the opportunity to host visiting students within the framework of the related project.

EMA is involved in other [research projects](#), that are part of the Innovative Medicines Initiative (IMI).

Between 2018-2019, the agency engaged in 13 new projects (seven IMI projects, five Horizon 2020 projects and one nationally funded project), either as consortium member as part of an Advisory board or hosting students.

TARGETED ENGAGEMENT



The Agency has targeted engagement with academia, learned societies and research groups in a range of areas, where they enable the Agency to move forward with its mission. These areas include:

- providing a forum for discussion and debate in the EMA scientific committees and working parties;
- organising and participating in scientific workshops and conferences;
- providing experts to steering committees of research projects and boards of learned societies;
- establishing and supporting networks of excellence;
- engaging in research initiatives of European or international health bodies;
- performing in-house data analysis, literature reviews and database studies in relation to the evaluation of medicines;

INNOVATION TASK FORCE (ITF)



The Innovation Task Force (ITF) is a multidisciplinary group that includes scientific, regulatory and legal competences. It provides a forum for early dialogue with applicants, in particular SMEs and academic sponsors, to proactively identify scientific, legal and regulatory issues linked to innovative therapies and technologies. Since the implementation of the academic framework, the ITF saw an increased in the number of IMI/ Horizon 2020 consortia applying for an ITF briefing meeting. This reflects a decision to proactively invite research consortia funded under the Horizon 2020 programmes, including IMI projects, to ITF briefing meetings.

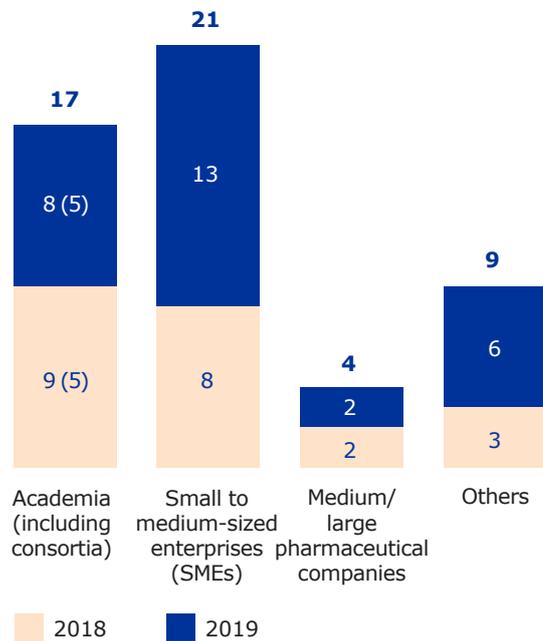


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ITF BRIEFING MEETINGS

2018
22

2019
29



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Stakeholder Engagement report 2018–2019: Engaging with patients,
consumers, healthcare professionals and academia
EMA/124131/2020

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