EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

EMA's biennial report on stakeholder engagement activities

2022-2023

An agency of the European Union

In memory of Noël Wathion

In remembrance of Noël Wathion (11 September 1956 – 12 August 2023).

Former Head of Stakeholders and Communication (2013-2016); deeply passionate and committed to public health; champion of transparency and public engagement.

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Foreword by Emer Cooke

Stakeholder engagement has always been and continues to be a pivotal part of the Agency's work, given the shared commitment to providing high quality, safe and effective medicines to patients and animals in Europe. The ongoing work to deliver on the European Medicines Agencies Network Strategy and Regulatory Science Strategy as well as our response to the Covid-19 crisis have clearly shown the importance of transparency and early engagement with all of EMA's stakeholders including the general public. Such proactive engagement was further pursued during the course of 2022 and 2023 to ensure that stakeholders' views are integrated into EMA's strategic activities (such as the implementation of the recent legislation extending EMA's mandate), and to enable knowledge sharing and learning from stakeholder experts on specific initiatives. In addition to bilateral interactions, promoting synergies through multi-stakeholder dialogue and cooperation has been a pivotal part of efforts to address complex issues around medicines, such as availability, crises preparedness and innovation.

Throughout 2022 and 2023, it has been a pleasure to witness many tangible examples of our stakeholders' dedication and to public and animal health. These are illustrated in this biennial report, which clearly highlights the many activities that EMA and EU national competent authorities have undertaken together with stakeholders, and I am confident that this enthusiasm and commitment will continue to grow for the benefit of EU citizens and public and animal health in the coming years.

Executive summary

Collaboration between the European Medicines Regulatory Network (EMRN) and its stakeholders is now well established and is at the cornerstone for ensuring that the needs of patients, healthcare professionals and others are taken into account when approving and monitoring medicines. It is also essential to help medicine developers, academics and innovators navigate the regulatory requirements and available support frameworks. Such collaboration was well illustrated in 2022, and is one of the learnings from COVID-19 and the response to the Mpox public health emergency. During the height of COVID-19, involvement of patients and healthcare professionals and discussions with industry not only in relation to the crisis response but also continued to support the development and evaluation of medicines in other areas (including for special populations and patients with rare diseases). Additionally, these stakeholders and academia contributed with their valuable perspectives to several initiatives, such as ACT EU, the implementation of agile governance, the establishment of the Industry Standing Group and various multi-stakeholder workshops. With the end of the Public Health Emergency of International Concern (PHEIC) for the disease caused by the coronavirus SARS-CoV-2, and EMA's business continuity plan in 2023, the focus shifted to resuming activities that had been put on hold during the COVID-19 emergency, such as face to face meetings with stakeholders. In the area of veterinary medicines, key contributions from EMA's stakeholder engagement activities have also been valuable for animal health, including activities implementing the recent amended veterinary regulation.

MULTISTAKEHOLDER APPROACH

During 2022 and 2023, stakeholder engagement activities followed a multi-stakeholder approach whenever possible, with the aim of working together to identify bottlenecks and find solutions to shared regulatory challenges. This complements the bilateral engagements that take place as outlined in the established frameworks for interaction with patients/consumers, healthcare professionals, industry trade organisations and academia.

The COVID-19 public health emergency illustrated the value of early dialogue and interaction between all stakeholders, and showed how open and constructive engagement has the power to improve regulatory outcomes. Early dialogue with developers facilitated the submission of more mature applications and more expedited scientific advice. Patients and healthcare professionals' representation on the EMA Emergency Task Force (ETF), as well as their active engagement through public meetings, proved to be of great added value. The ACT EU multistakeholder platform was a tangible example of all stakeholders contributing with their perspectives to discussions on challenges and opportunities in the clinical trial environment. Integrating the voice of all concerned stakeholders in the implementation of the legislation that extended EMA's mandate was another important occasion to identify stakeholders' priorities on topics such as crisis management and preparedness, as well as monitoring and ensuring availability of medicines and medical devices. The multifaceted dialogue linked to medicines innovation aspects was also key, such as patient-centred clinical trials, understanding new technologies, and the use of artificial intelligence for medicines regulatory purposes.

Embedding early multi-stakeholder engagement alongside activities targeting single stakeholder groups is expected to continue in the coming years, given the importance of addressing bottlenecks and fostering mutual understanding of key topics impacting public

health, as also outlined in the European medicines agencies network strategy to 2025 – Mid-point report to Q2 2023. Fundamental will be the dialogue on the effective implementation of the revised EU pharmaceutical legislation, which will represent a key milestone in addressing challenges such as ensuring medicines availability and adapting the EU medicines regulatory system to an evolving scientific and regulatory landscape.

ENGAGEMENT WITH PATIENTS AND CONSUMERS

The last two years saw a key achievement in EMA's engagement with patients and consumers, with the adoption of the CHMP early contact with patient organisations as an established methodology. This followed a successful pilot with orphan medicines in 2021-2022 and adds to the existing methods for gathering patient input into medicines development and evaluation. The methodology was extended to all (non-orphan) medicines in 2022. The added value of patient engagement in EMA's scientific advice and its impact on regulatory decision-making has been documented in a peer-reviewed scientific article. During 2022-2023, EMA also continued its collaboration with the US FDA through the Patient Engagement Cluster and held joint meetings between EMA's Patient and Consumer Working Party and the FDA Cluster on issues of joint interest.

Another recent priority area for the Agency is Patient Experience Data (PED), which refers to data reported directly by patients about their own disease experience and preferences for treatment outcomes. Although there has been much progress in the EU in recent years, PED are still not systematically included in all aspects of medicines development and regulation. The EU Medicines Regulatory Network identified the need to reinforce patient relevance in evidence generation as a key priority to increase patient access to medicines. This requires a focus on the development of standards for designing, conducting, analysing and reporting relevant studies that incorporate robust and meaningful patient experience data for regulatory submissions. In 2022 the Agency organised a multi-stakeholder workshop on PED and in 2023 launched an EU Network-wide initiative to prepare a reflection paper on the EU approach to the collection, analysis and use of PED in regulatory decision-making, with involvement of patient representatives as well as other stakeholders. This work is also related to EMA's work on big data and real-world data, where patients and consumers also continue to be closely involved.

EMA continues to promote transparency as a key commitment to stakeholders and the general public. Future activities with patient and consumer organisations will include exploring how to adapt the public assessment report template to better reflect patient and healthcare professional input on EMA assessments, such as that received via the CHMP early contact. Methodologies for integrating patient input earlier will be explored concurrently with the PED initiative, particularly with regard to the evaluation of the benefits and risks of medicines. Engagement on information and communication about medicines and vaccines will be strengthened, in particular on antimicrobial resistance and in tackling mis- and disinformation. Finally, as training remains critical in supporting the capacity of patient representatives and organisations to participate in EMA's work, the Agency will map training needs, with a particular focus on emerging complex topics such as patient experience data, real-world data and artificial intelligence.

ENGAGEMENT WITH HEALTHCARE PROFESSIONALS

In June 2023, EMA marked 10 years of successful operation of the Healthcare Professionals Working Party (HCPWP). In addition, the healthcare professional organisations' Policy

Officers Group continued to bring a valued complement to the HCPWP, particularly when identifying areas where deeper discussion with regulators is needed. In addition, the CHMP early contact with patient organisations was extended to healthcare professional organisations.

Further to these key achievements in EMA's engagement with healthcare professionals, EMA initiated a reflection to review the current framework of interaction with healthcare professionals and their organisations in order to incorporate learnings gained since the framework's last revision in 2016. Looking ahead, we will be focusing on how best to engage with new generations of healthcare professionals by establishing more structured collaboration with their organisations, students' associations and academia. Attention will be also given to other areas for engagement related to public health, clinical practice and information on medicines, including for example shortages, electronic product information, antimicrobial resistance, mis- and disinformation, and the European Vaccination Information Portal. We will continue to promote awareness of existing engagement methodologies and regulatory tools supporting medicines development and evaluation, and further consolidate healthcare professionals' involvement in the full spectrum of EMA's activities, including the implementation of new legislation.

ENGAGEMENT WITH ACADEMIA

Much of the scientific discovery and knowledge that eventually gives rise to medicines comes from research by academia. Maintaining close interaction with academic stakeholders is essential to ensure that regulators can support innovation in medicines development and in regulatory science that underpin the Agency's work. EMA's interactions with researchers and developers from the academic sector have continued to grow during the period covered by this report. For example, there has been an increase in the number of applications for PRIME status and orphan medicine designation, as well as an increase in the number of scientific advice preparatory meetings. Additionally, two pilot programmes on drug repurposing (2022) and ATMPs development (2023) for academic stakeholders were launched. Within EMA, an academia workstream was consolidated to provide a more tailored support to the academic sector. Academia briefing meetings, providing a venue for bidirectional learning, are now part of the Agency's support to these stakeholders; around 20 academia briefings, to facilitate the exchange of information for medicines development and regulatory science, were held during the reporting period. This is in addition to the 42 Innovation Task Force briefing meetings held with academic stakeholders and focusing on the provision of regulatory insight for innovative developments. EMA's collaboration in external research projects yielded over 30 peer-reviewed publications in the reporting period. The Agency also collaborated with funding organisations such as the European Commission research executive agencies and national funding organisations to make recommendations for research funding proposals and to provide regulatory and scientific input on specific elements in planned funding calls.

Other activities to strengthen collaboration with academia include the publication of audiovisual training material in collaboration with academic organisations to disseminate knowledge on orphan medicine designation and advanced therapies. Moreover, EMA's first in-house Academia Info Day was successfully held on 9 November 2023, with high attendance and much interest from the sector.

EMA continues to work on gaining a better understanding of the sector and develop further support initiatives tailored to the needs of academia and will also promote, including by

liaising with funding organisations and through regulatory interactions for funded projects. The Agency is also expanding interactions with the academic sector developing medicines, technologies or drug development tools. Additionally, EMA will further support external research, particularly where it addresses the Regulatory Science Research Needs.

ENGAGEMENT WITH INDUSTRY EU TRADE ORGANISATIONS

The pharmaceutical industry is another important stakeholder for the Agency, given its key role in medicines research and development, manufacturing and supply. During 2022-2023, EMA engaged in regular dialogue with human and veterinary industry EU trade organisations through the established EMA-industry platforms and bilateral meetings. These have fostered mutual understanding of key elements in the medicines lifecycle and identified sector-specific needs to gain efficiency in regulatory processes. Specifically, several initiatives continued to support small-, medium sized- enterprises (SME), through the SME office, targeting core business activities with companies through the established mechanisms of interaction (e.g., scientific advice, PRIME scheme, pre-submission meetings, etc.).

Industry stakeholders had the opportunity to share their specific perspectives through an open dialogue, flagging needs and challenges posed by the evolving global and European scientific, technical and regulatory environment. In addition, existing industry stakeholder engagement mechanisms were further reinforced in June 2022 with the establishment of the Industry Standing Group (ISG). This new group was initially established to facilitate the implementation of Regulation (EU) 2022/123, reinforcing EMA's role in crisis preparedness and management of medicinal products and medical devices. This provided a platform for industry stakeholders to engage with EMA and allowed the medical device and *in vitro* diagnostic industry sectors, as well as the whole industry supply chain representatives to be incorporated into EMA activities as new stakeholders following its extended mandate. These newcomers will play an essential role in successfully facilitating new monitoring and reporting processes, as well as the development of databases, for example for the future management of crisis shortages. Based on a successful first year, the scope of ISG was further extended in 2023 to address key topics of strategic importance and mutual interest to EMA and across industry stakeholders. In this respect, ISG is expected to continue providing a forum for engagement on topics such as the implementation of EMA's extended mandate, the implementation of the EU pharmaceutical legislation, the use of artificial intelligence for smart regulation, and any other strategic topic related to EMA and its international activities.

CONCLUSIONS

The past two years saw an increase in stakeholder interactions and critical input in response to the unprecedented challenges posed by the COVID-19 pandemic. This report recognises how crucial stakeholder input was in articulating EMA's response to the crisis. They supported specific considerations such as discussions on shortages management, vaccines, vaccine hesitancy, etc., while helping the Agency convey public health messages directly to patients, healthcare professionals and EU citizens. Stakeholders also made important contributions to discussions on challenges and opportunities brought by the implementation of new legislation (i.e., the Clinical Trial Regulation, the Veterinary Medicines Products Regulation and EMA's extended mandate), the development of important initiatives (such as Accelerating Clinical Trials in Europe (ACT EU), big data, Agile transformation, and patient experience data).

There are many opportunities ahead of us such as the reform of the EU pharmaceutical legislation, the use of artificial intelligence in medicines for smart regulation, the fight against antimicrobial resistance, and the global health threat still posed by COVID-19 and other infectious diseases, etc). In view of this, collaboration with stakeholders will become even more pivotal as the Agency continues its work in medicines regulation. In all these areas, transparency, timely dissemination of information, cooperation and dialogue between all the concerned parties have been and will remain key to providing EU citizens with the science and data underpinning EMA decisions and reinforcing their trust in the scientific outcomes of the EU Network.

The work presented in this report would not have been possible without the input and support provided by patients, consumers, healthcare professionals, academia and industry stakeholders. The Agency is grateful for the ongoing collaboration and looks forward to more engagement in the years to come.

Multistakeholder engagement

"Dialogue among all stakeholders is vital in an era marked by transformative changes in medicine, science, technology and society. Lessons learnt from the COVID-19 pandemic coupled with the extension of EMA's mandate have led to the integration of an earlier, multi-stakeholder approach to engagement on key activities and in delivering on our strategic priorities."

Melanie Carr, EMA's Head of Stakeholders and Communication

Although EMA has routinely organised multi-stakeholder discussions for key topic areas, by design the latest scientific, technological and regulatory advances require earlier involvement of all relevant parties. During 2022 and 2023, EMA systematically engaged with stakeholders at a multi-stakeholder level on strategic areas concerning both product-related activities and public health priorities. Early engagement with all stakeholders to understand each other's perspectives, promote dialogue and facilitate cooperation were recurring themes that will continue to be promoted as much as possible in the future. In 2022-2023, multi-stakeholder engagement targeted priority areas such as crisis management and preparedness, ensuring availability of medicines and medical devices, progressing in regulatory science areas such as patient experience data, promotion of research, innovation and development.

Ensuring availability of medicines in crisis and outside crises

At the core of EMA's activities lies the need to ensure timely access to medicines for citizens and animals in the EU/ EEA. EMA therefore pursued engagement with stakeholders within the context of crisis preparedness and monitoring of shortages.

Crisis preparedness and management

The Agency engaged with stakeholders through various forums on the main activities arising from EMA's extended mandate, which reinforced EMA's role in crisis preparedness and management, and from the lessons learnt from COVID-19.

- During the COVID-19 pandemic the Emergency Task Force (ETF) (previously known as EMA's Pandemic Task Force) played a key role in facilitating faster access to vaccines and treatments.[1] Under the extended mandate legislation, ETF formally became an advisory and support body on medicines during public health emergencies as well as during times of preparedness. In recognition of the contribution of civil society, experts from the Patients' and Consumers' Working Party (PCWP) and Healthcare Professional's Working Party (HCPWP) were included as members of ETF.
- Following discussions at the European Medicines Agency / Emergency Task Force and European Commission workshop on lessons learned on clinical trials in public health emergencies, a formal priority action was established under the ACT EU initiative to tackle certain aspects of clinical trials during emergencies, including promoting alignment and collaboration across Member States, the Emergency Task Force and ethics committees. The ACT EU multi-stakeholder platform will play an important role inr discussing and monitoring future progress of the initiative.

Availability and accessibility of medicines

Medicine shortages or other availability issues can have a potentially serious impact for patients and animals. During 2022 and 2023, EMA and the European Medicines Regulatory Network (EMRN) progressed on activities to ensure the availability of authorised medicines, including during times of crisis such as public health emergencies or other major events.

Month/Year	Activities
March 2022	The regulation reinforcing EMA's role in crisis preparedness and management of medicinal products and medical devices became applicable, giving EMA new responsibilities for monitoring medicine shortages that might lead to a crisis situation, as well as reporting shortages of critical medicines during a crisis. The Agency also became responsible for the coordination of responses of EU/EEA countries to shortages of critical medical devices and in-vitro diagnostics in public health emergencies. In this context EMA has been working together with stakeholders to effectively implement this new mandate.
April 2022	The key changes brought by the new regulation and their impact on stakeholders were discussed in a dedicated multi-stakeholder workshop on EMA's extended mandate. The importance of cooperation and continuous engagement was recognised and considered to be essential for successful crisis preparedness and management.

[1] https://bpspubs.onlinelibrary.wiley.com/doi/10.1111/bcp.15567

Month/Year	Activities
March 2022	The Executive Steering Group on Shortages and Safety of Medicinal Products (MSSG) was established under Regulation (EU) 2022/123 to monitor shortages. The MSSG's role is to ensure a robust response to major events or public health emergencies and to coordinate urgent actions on the supply of medicines within the EU. The inclusion of observers from the EMA's PCWP and one from its HCPWP highlights the pivotal role of patients and healthcare professionals in shortages discussions.
June and August 2022	Following the MSSG's adoption of the lists of critical medicines for COVID-19 and Mpox public health emergencies, respectively, marketing authorisation holders were required to provide regular updates to EMA, including data on potential or actual shortages, available stocks, and forecasts of supply and demand. In addition, Member States provided regular reports on estimated demand for critical medicines at national level.
July 2022	The EMA published a good practice guidance for patient and healthcare professional organisations on the prevention of shortages of medicines for human use. The guidance, developed in collaboration with patients and healthcare professional representatives, outlines key principles and provides examples of good practice to support these stakeholders in preventing and managing shortages of human medicines: Towards better prevention of medicine shortages in the EU.
January 2023	At the beginning of 2023, focus was placed on monitoring and managing the shortages of antibiotics, especially paediatric formulations, which was caused by a surge in respiratory infections. EMA worked closely with manufacturers, patients and healthcare professional representatives to manage the situation, including raising awareness of the importance of prudent use of antibiotics as outlined in the Joint statement by Executive Steering Group on Shortages and Safety of Medicinal Products (MSSG) on shortages of antibiotic medicines

Month/Year	Activities
March 2023	During the Heads of Medicines Agency (HMA)/EMA multi-stakeholder workshop on shortages, stakeholders discussed the activities of the HMA/EMA Task Force on shortages and were invited to reflect on how each stakeholder group can contribute to addressing availability issues and enable the switch from a reactive (management of shortages) to a proactive thinking (prevention of shortages). Focussed, timely and effective communication was recognised as a key element for shortages preparedness and management in addition to adequate prevention plans.
2023/2024	To ensure preparedness for the 2023/2024 autumn/winter season, a joint EMA-HERA (Health Emergency Preparedness and Response Authority) exercise was undertaken to monitor the supply and demand of a subset of antibiotics used to treat respiratory infections. In this context, through the coordination of industry EU trade organisations, marketing authorisation holders provided data on supply forecasts and production capacity, which were fundamental in enabling the MSSG to publish a series of recommendations in July 2023 for all involved stakeholders in order to ensure availability of these antibiotics.
October 2023	Further steps to address critical shortages of medicines in the EU were taken with the publication of the MSSG toolkit providing recommendations for monitoring supply and demand and the establishment of the solidarity mechanism allowing Member States to support each other in the face of a critical medicine shortage.
December 2023	The Union list of critical medicines and related Q&A document were published. This list contains human medicines whose continued supply is considered a priority in the EU to avoid serious harm to patients and help healthcare systems function.

Stakeholders' expertise was sought for the development and testing of the European Shortages Monitoring Platform (ESMP) and the Critical Medical Device Shortages (CMDS) reporting systems, given their fundamental impact on the current reporting requirements and practices. In both cases the Scaled Agile Framework (SAFe) mindset was used, enabling subject matter experts nominated by industry EU trade organisations to support the technical discussions. Ensuring data interoperability to enable the exchange of data between both industry and NCA systems and the ESMP and CMDS was also a key requirement and is currently under discussion.

Month/Year	Activities
July 2023	A CMDS minimum viable product was developed in close cooperation with various stakeholder groups, including economic operators, Member States, and notified bodies. This enabled the first release in July 2023.
November 2022	EMA launched a pilot project allowing eligible patient, consumer and healthcare professional organisations to report specific shortages to EMA. The pilot initially ran for six months and collected reports of shortages of 33 active substances. The possibility of reporting is now continuing for shortages affecting more than two Member States involving a medicine to treat a disease with serious implications for health, where no suitable alternative exists and where the shortage is not already listed in the national shortage catalogue.

Key achievement

Crisis preparedness and management activities have progressed with activities facilitating large and multinational clinical trials during public health emergencies now included in ACT EU workplan; with progress on the development of ESMP and CMDS reporting and monitoring databases and with the publication of the MSSSG toolkit and Union list of critical medicines.

Data analytics, digital tools and digital transformation

EMA continued its efforts to promote multistakeholder engagement on the development of innovative medicines within the EU/EEA region by enabling dialogue on several aspects of research and innovation such as clinical trial improvement, big data analysis, and research and innovation, especially in the fields of oncology and unmet medical needs.

Accelerating Clinical Trials in the EU (ACT EU) initiative

ACT EU is an initiative launched by the European Commission, EMA and HMA in January 2022. The vision of ACT EU is to make the EU an attractive region for clinical research, enabling more impactful clinical trials with seamless coordination between regulators, ethics committees and relevant stakeholders. During 2022 and 2023, several multi-stakeholder activities took place to promote dialogue among stakeholders and initiate discussion on key priority areas such as the implementation of the Clinical Trials Regulation (CTR) and its business tool, the Clinical Trials Information System (CTIS), the launch of a scheme to support academic sponsors conducting large multi-national trials, and the creation of a multi-stakeholder platform to facilitate of dialogue among stakeholders and regulators.

Month/Year	Activities
July to September 2022	A survey was launched to understand the issues faced by sponsors submitting trials under the CTR, resulting in a report summarising the findings and subsequent actions taken by key network groups. A follow-up clinical trial sponsor survey was launched in September 2023 to monitor progress in the implementation of the CTR.
October 2022	Stakeholders participated in the ACT EU multi-stakeholder meeting on decentralised clinical trials to discuss related topics. The valuable input gathered was reflected in the Recommendation paper on decentralised elements in clinical trials.
June 2023	The ACT EU multi-stakeholder platform kick-off workshop marked the formal beginning of multi-stakeholder interactions and discussions about key aspects of clinical trials. This event followed the public stakeholder consultation on ACT EU multi-stakeholder platform (ACT EU MSP) participation and priorities for discussion, which raised the need to bring together all key players at an early stage. During the workshop, the model for the creation of a multi-stakeholder platform advisory group (MSP AG) was agreed, enabling stakeholders to advise on priorities for discussion and actively contribute to ACT EU deliverables. A call for stakeholder representatives was launched in October 2023, with the establishment of the MSP AG foreseen in early 2024. Another important outcome of the workshop was the agreement on the need to have a dedicated priority action (Clinical trials in public health emergencies), focusing on how to facilitate large and multinational clinical trials in the EU to promptly tackle public health emergencies.
October 2023	Publication of information on clinical trials in the EU/EEA is important to build trust and support clinical research. The public stakeholder consultation launched in May 2023 collected stakeholders' views on how to enable timely transparency in CTIS. The consultation resulted in the publication of the revised CTIS transparency rules in October 2023.
July 2023	Stakeholders participated in a workshop on transitioning trials to the CTR/CTIS. Feedback from this event informed the revised best practice guide and annex from HMA's Clinical Trials Coordination Group (CTCG) and the dedicated Guidance for the transition of clinical trials from the Clinical Trials Directive to the Clinical Trials Regulation which was published by the European Commission.
July/2023	Another area of focus was supporting the implementation of ICH E6(R3) Good Clinical Practice, including through the ACT EU Multi-stakeholder Workshop on ICH E6 R3. The PCWP and

Month/Year	Activities
	HCPWP were closely involved and submitted a joint contribution to the ICH public consultation – the culmination of a long collaborative process initiated in 2020 when a workshop with PCWP and HCPWP was organised on this topic.
November 2023	ACT EU aims to facilitate the development of aligned clinical trial guidance across the EMRN, resulting in high impact guidance documents implemented in practice. A multi-stakeholder methodology workshop offered participants the opportunity to understand different stakeholder perspectives on the development of clinical trial methodology guidance, discuss the status of guidance on priority clinical trial methodology topics and identify the need for new guidance or updates to the existing guidance for the priority methodology topics.

Key achievement

Stakeholders now benefit from the ACT EU multi-stakeholder platform as a vehicle for clinical trials stakeholders and regulators to come together, voice their views and collaborate to improve the clinical trials environment for European patients and citizens.

Use of data for evidence generation

BIG DATA

At the core of the big data initiative lies effective use of real-world evidence in regulatory decision-making to support medicines development and authorisation. Implementation of the Big Data initiative is guided by the Big Data Steering Group (BDSG) workplan, which is updated annually.

The update to the Big Data workplan in July 2023 reinforced collaboration with patients organisation representatives. The plan foresees discussions to explore use cases for patient experience data to better support regulatory decision-making, a workshop on registries and gathering patients' training needs. Patient representation continued in the revised mandates of the BDSG and DARWIN EU® Advisory Board that took place in 2023.

In 2022 and 2023, stakeholders had the opportunity to contribute to workplan discussions during the annual big data stakeholder forums and through events on dedicated topics, such as DARWIN EU, real world data and novel methodologies.

Stakeholders were also invited to participate in the following public consultations:

- Reflection paper on the use of artificial intelligence in the lifecycle of medicines
- Good Practice Guide for the use of the Metadata Catalogue of Real-World Data Sources
- Data Quality Framework for EU medicines regulation
- ICH Reflection paper on proposed international harmonisation of real-world evidence terminology and convergence of general principles regarding planning and reporting of studies using real-world data, with a focus on effectiveness of medicines

PATIENCE EXPERIENCE DATA

Among the priorities of the European Medicines Agencies Network Strategy and the Regulatory Science Strategy is the systematic incorporation of patient experience data (PED) in medicines development and evaluation. Patients' and carers' perspectives on the use, benefits and risks of medicines are of great value to decision-making by EMA and the EU Regulatory Network and are also of importance to downstream decision-makers. Although there has been much progress in the EU in recent years, PED are still not systematically included in all aspects of medicines development and regulation.

In view of this and given the value of PED, a multi-stakeholder workshop was held on the topic in September 2022. The workshop acted as a catalyst to initiate work in this area based on the challenges identified at the workshop. EU regulators have since prioritised two actions which were initiated in 2023. The first responds to the request for regulatory guidance for companies and patient groups collecting and/or analysing PED or wishing to develop PED methodologies. To respond to this need, a reflection paper will be developed to provide an EU framework on how to approach PED generation, collection, storage and analysis of PED. Both at and after the workshop patients, consumers and healthcare professionals contributed extensively to discussions and will also be involved in the development of the reflection paper.

The second action focuses on increasing transparency on how PED are used and considered during the benefit-risk assessment of medicines. During 2023, EMA liaised regularly with stakeholders on PED, both during PCWP/HCPWP meetings (June 2023, November 2023) and in bilateral meetings with industry (June 23 and November 23 industry platform meetings). In October 2023, EMA also published an updated Day 80 CHMP clinical assessment report template, which enables patient engagement such as patient-reported outcomes to be better captured and presented during the assessment. EMA will continue working in these two areas during 2024.

ARTIFICIAL INTELLIGENCE

The use of artificial intelligence (AI) to enable smart regulation has been an important topic in discussions with stakeholders. The EMA AI workshop – Smart regulation in a rapidly evolving world held in November 2023 allowed multi-stakeholder engagement with industry, academia, patients and healthcare professional stakeholders on AI developments in technology, policy and potential applications in medicines regulation.

VETERINARY BIG DATA

The Veterinary Big Data initiative aims to enable the use of new digital technologies and prepare for future changes relating to big data in regulatory decision-making for veterinary medicines.

In May 2022, EMA and HMA adopted the EMA/HMA veterinary big data strategy for 2021-2027 and, a year later, endorsed the EU veterinary big data workplan. Implementation of the veterinary big data initiative is driven by this multi-annual workplan which transposes the strategy into actionable workstreams and sets out milestones and timelines for the veterinary big data initiative from 2023 to 2025. In 2022 and 2023, stakeholders had the opportunity to contribute to workplan discussions during the annual big data stakeholder forums and through events on dedicated topics.

Medicines innovation and development

During the course of 2023, a number of events were organised to foster the dialogue on regulatory support and evidence generation for innovative medicines and to enable the transfer of knowledge gained through the COVID-19 crisis to therapeutic areas beyond vaccines.

Month/Year	Activities
January 2023	EMA and the European Innovation Council (EIC) hosted an Info Day: Regulatory support for the development of innovative medicines and technologies. The event was an opportunity for researchers and SMEs active in research and innovation in the pharmaceutical and med-tech sectors to learn more about the regulatory support available for innovative medicines.
February 2023	All stakeholders had the opportunity to discuss the current state-of-the-art RNA technologies, future developments and guidance needed at the Regulatory and scientific virtual conference on RNA-based medicines. The initiative focussed on continuing the dialogue with stakeholders on emerging RNA technologies, which was initiated during the COVID-19 pandemic.
September 2023	The dialogue on research and development of innovative medicines and related technologies & methodologies in the EU was further promoted by the EU Innovation Network (EU-IN) and the Spanish presidency of the Council of the European Union with the multi-stakeholder event entitled Shaping a European innovation ecosystem: EU-Innovation network multi-stakeholder meeting. The event focussed on identifying gaps in regulatory science and how to move forwards and further catalyse the development of innovative medicines in the EU. The dialogue on strengthening innovation and supporting the translation of innovative biopharmaceutical and medical technology developments further continued in November 2023 with the European incubators and Technology Transfer Offices

Month/Year	Activities
	(TTOs) through the Strengthening life-sciences innovation across Europe: EU-Innovation Network conference.
November 2023	Organisations representing academia, industry, regulators, healthcare professionals and patients joined a workshop to discuss how to best design clinical trials that generate evidence to support the treatment and prevention of long COVID and post-acute sequalae condition. Workshop on generating clinical evidence for treatment and prevention options for long-COVID and post-acute sequelae condition (PASC)
September 2023	The Agency held a live event on LinkedIn to discuss how human and animal health are closely interlinked, highlighting the need for innovation in veterinary medicines. EMA's Head of Veterinary Medicines Division & Deputy Executive Director, Dr Ivo Claassen, responded to questions on innovation, collaboration with academia, but also on how to combat antimicrobial resistance and manage the impact of veterinary medicines on the environment. Tomorrow's veterinary medicines for healthy animals and humans

MEDICINES FOR CHILDREN AND ADOLESCENTS

Multistakeholder engagement is of great importance when discussing the role of research and innovation in helping to make more medicines available for use in children and adolescents.

Month/Year	Activities
January 2023	Throughout 2022 and 2023, EMA continued to support the activities of the European Network of Paediatric Research (Enpr-EMA) with annual events to bring together international experts, including academia, the pharmaceutical industry and healthcare professionals, as well as parents, carers and patient representatives. Ongoing priority activities include strengthening the role of paediatric research nurses, developing quality criteria for paediatric clinical trial sites, and improving paediatric patients' access to clinical trials across borders. Furthermore, work is ongoing to support academic sponsors as part of the ACT EU initiative, as well as discussions on best practices for patient recruitment and involvement of patients and young people in clinical trial planning.

The Accelerate platform is another key initiative enabling discussions with stakeholders at international level on how to promote childhood cancer drug development. In 2022 and 2023, in addition to the annual conferences, the group hosted several strategy forums where stakeholders discussed various topics and reached consensus on future development. One of these forums, which is held twice a year, focuses on either a disease or class of products and alternates between the US and Europe. This forum brings together all stakeholders, with the recent addition of health technology assessment (HTA) bodies and aims to ensure that medicines development for children with cancer is targeted, efficient and addresses public health needs. An impactful outcome of one of the latest forums, the Mitogen-Activated Protein Kinase (MAPK) Forum was a mini symposium hosted by the FDA on functional endpoints in paediatric low-grade glioma to which EMA actively contributed. This symposium resulted in a publication (Considering Functional Outcomes as Efficacy Endpoints in Paediatric Low-Grade Glioma Clinical Trials: An FDA Educational Symposium), which is expected to be published in Q1 2024 and invites further interactions with global regulators on the development of functional endpoints most relevant to patients. As outlined in the European regulatory strategy for supporting childhood cancer therapy developments published in 2022 and the Paediatric Strategy Forum for medicinal product development of DNA damage response pathway inhibitors in children and adolescents with cancer: ACCELERATE in collaboration with the European Medicines Agency with participation of the Food and Drug Administration published in 2023, the forums are considered to be a key pillar in advancing efforts to facilitate innovation based on evidence in rare diseases such as paediatric oncology.

Implementation of the EU Veterinary Medicinal Products Regulation

On 28 January 2022, the Veterinary Medicinal Products Regulation (Regulation (EU) 2019/6) became applicable. Throughout the year, the Agency organised support activities for stakeholders who were directly impacted by the regulatory changes introduced by this regulation. In the following year, EMA expanded its focus to include other stakeholder groups.

In September 2023, the Agency held the first-ever Veterinary Awareness Day, designed to provide an overview of the Agency's work in veterinary medicines science and regulation in the EU and to give an insight into how related activities aim to benefit animal health and welfare.

Other engagement activities

All stakeholders registered in the EMA stakeholders database were engaged via targeted communications to raise awareness on relevant published news and to promote participation in other events, public consultations, surveys and calls for expression of interest. These were important opportunities for stakeholders to further contribute their perspective on initiatives, guidance development and/or revisions linked to both strategic and scientific topics. Such topics included the European Commission's impact assessment on the reform of the EU pharmaceutical legislation, the utility of safety communications on COVID-19 vaccines, antimicrobial sales and use in animals at the EU level, the update of veterinary guidance, quality aspects of mRNA vaccines, use of AI, and the EU medicines agencies reflections on the lessons learned from COVID-19.

Overall, during the course of 2022 and 2023, expert representatives from civil society, academia, patient organisations, healthcare professionals' organisations and industry EU trade organisations were given opportunities to share their perspectives on several topics and initiatives.

Targeted engagement with patients and consumers

"Engagement with patients and consumers is a cornerstone of EMA's mission to protect public health and critical to fostering innovation informed by patients' needs and experiences. Inclusion of patients' perspectives leads to better quality and outcomes of regulatory processes, supports transparency and increases trust."

Marko Korenjak, PCWP Co-Chair

Patients contribute to EMA's work in a number of ways – both collectively through eligible organisations and the Patients and Consumers' Working Party (PCWP), and as individual experts. In December 2023, there were a total of 43 eligible patient and consumer organisations. The PCWP comprised 22 member organisations. The number of patient/consumer representatives with a valid declaration of interests in the database of European Experts was 144 at the end 2023; these individuals represent patient and consumer experts who can be called upon at any particular moment to participate in EMA activities.

There are two patient representatives on the Agency's Management Board, and patients are members of the following scientific committees:

- Committee for Orphan Medicinal Products (COMP) – 3 members
- Paediatric Committee (PDCO) – 3 members and 3 alternates
- Committee for Advanced Therapies (CAT) – 2 members and 2 alternates
- Pharmacovigilance Risk Assessment Committee (PRAC) – 1 member and 1 alternate.

Patients and consumers are also represented in several EMA working groups and task forces, which include the Medicines Shortages Steering Group (MSSG), Medical Devices Shortages Steering Group (MDSSG) and the Emergency Task Force (ETF), as part of EMA's extended mandate. In addition they are involved in the DARWIN EU Advisory Board, the Big Data Steering Group (BDSG), the Drafting Group on Digital Support Tools to Risk Minimisation Measures, the Advisory Group on Raw Data, ENCePP Steering Group, Enpr-EMA, QRD

working subgroup, PRAC multistakeholder working group on digital Support Tools to Risk Minimisation Measures (RMM) and the ACT EU Multistakeholder Advisory Group.

Updated engagement framework: EMA and patients, consumers and their organisations

The framework of EMA's interaction with patients' and consumers' organisations was originally adopted by EMA's Management Board in 2005. As the experience and methodologies of engagement have evolved with experience and collaboration, new legislation, advances in science, and crisis situations, the framework was revised and published in early 2022. The updated framework reflects EMA's relationship with its patient and consumer stakeholders and enables the Agency to obtain their valuable input, increase transparency and build trust in EMA and the EU regulatory system. The title change from 'interaction' to 'engagement' also reflects the importance of the mutual exchanges between the Agency and the patient stakeholders.

Patients' and Consumers' Working Party

The Patients' and Consumers' Working Party (PCWP) provides a platform for exchange of information and discussion of issues of common interest between EMA and patients and consumers.

Table 1 provides an overview of the topics in which the PCWP was involved during 2022 and 2023. Many of the meetings are held in conjunction with the corresponding HCPWP as several subjects covered are of mutual interest and relevance. This is not only consistent with our multistakeholder approach but also results in better outcomes.

Table 1: PCWP involvement during 2022 and 2023

2022	2023
March	March
ACT EU • Big data • Electronic product information • EMA's extended mandate • Advanced therapy medicinal products (ATMPs)	EMA working party reorganisation • Medical devices • Clinical trials • Biosimilars and interchangeability • New EMA policy on multilingualism
June	June
New mandate and introduction to new members • Clinical trials • COVID-19 updates • Big data • Creation of joint drafting group on ICH E6 guidance on good clinical practice	Pharmacovigilance • Clinical trials • DARWIN EU • Electronic product information • Medicine shortages • Nitrosamines

2022

September

Update on COVID-19 • Big data • Use of EMA communications • Election on new co-chairs

November

Patient experience data • medicine shortages • HTA activities • Antimicrobial resistance

2023

September

Involvement of patient and HCP representatives in various EMA groups • Updates on crisis preparedness • Big data • Medicine shortages • Clinical trials • EMA communications

November

Availability and accessibility of medicines • EMA data related initiatives and digitalisation • Regulatory science and innovation • Communications • Members' voice

Election of new PCWP co-chair

In November 2022, Marko Korenjak, President of the European Liver Patient Association (ELPA), was elected as co-chair of the PCWP. This marked the beginning of his mandate. This exceptional election followed the September elections when Marilena Vrana from the European Heart Network (EHN) was elected as co-chair but had to step down due to a change in her professional activities. The role is shared with the EMA co-chair, Juan Garcia Burgos, Head of Public and Stakeholder Engagement at EMA.

Engagement on medicines safety issues

Patient organisations regularly participate in stakeholder meetings requested by the Pharmacovigilance Risk Assessment Committee (PRAC), the EMA committee tasked with assessing and monitoring the safety of human medicines. In 2023, EMA held two virtual stakeholder meetings on valproate-containing medicines, which are used to treat epilepsy and bipolar disorder, and in some EU countries to prevent migraine. The meetings aimed to better understand the potential barriers and enablers for successful implementation of the risk minimisation measures attached to these medicines. For more details, please see page 34.

Interactions with FDA supporting patient engagement

EMA continued its collaboration with FDA to support patient engagement through various activities. The Patient Engagement Cluster, established in 2016 with the aim of both agencies

to share initiatives and experiences on best practices for engaging patients in discussions on medicines, continued to meet virtually on a quarterly basis.

In addition, the PCWP meets yearly with the PEC, which is a joint project between the FDA and the Clinical Trials Transformation Initiative (CTTI). This allows the two patient groups to discuss transnational topics of public health interest, such as patient information, communication and youth engagement. In 2022, the topics of these joint meetings included lessons learnt from COVID-19 and emerging issues in patient engagement, while in 2023 the focus was on decentralised clinical trials.

Finally, in collaboration with the FDA Oncology Center of Excellence, a 'Conversations on Cancer' public panel discussion was held on 19 October 2023. The panel featured speakers with a range of perspectives on breast cancer and the discussion addressed the experience of living with metastatic breast cancer. EMA was pleased to join this international collaboration, which highlighted the day-to-day and year-to-year experience of patients living with metastatic breast cancer. Through this collaboration, EMA also aimed to identify common threads and/or distinctions among patients and healthcare providers in the US and in Europe. Additional information can be found under events of interest at Cancer | European Medicines Agency (europa.eu).

Bilateral meetings

Bilateral meetings can be organised upon request by an eligible patient or consumer organisations to discuss issues of common interest. During these meetings, EMA aims to listen and understand the stakeholder's areas of concern and provide additional information or clarifications. The meetings complement discussions at the level of the PCWP and provide an opportunity to go in depth on a specific organisation's areas of concern or subject matter. Confidential and product-specific discussions are out of scope.

In 2022 bilateral meetings were held with the Thalassaemia International Federation (TIF) and the European Haemophilia Consortium (EHC). In 2023, bilateral meetings were held with the European Consumer Organisation (BEUC) and TIF. In 2023 the Agency initiated the publication of the agendas and minutes of these bilateral meetings.

Contributions to patient organisations' meetings

As described in the engagement framework, to raise awareness on EMA's activities and facilitate patient and consumer organisations' participation in the Agency's work, there is a regular contribution to trainings and conferences organised by patient and consumer organisations.

Selected examples include:

- EURORDIS Open Academy
- Vitiligo International Patient Organizations Committee
- Myeloma Platform Europe Advocate Development Program
- European Federation of Allergy and Airways Diseases Patients' Associations
- International Alliance of Patient Organisations
- European Huntington Association
- Osteoarthritis Foundation International

- Digestive Cancers European Medicines Agency
- European Patients' Academy on Therapeutic Innovation (EUPATI)
- Familial Hypercholesterolaemia (FH) Europe

Patient contribution to the evaluation of medicines

Whenever possible, EMA involves patients as individual experts in expert meetings such as scientific advice and scientific advisory group or ad hoc expert group meetings. EMA also involves patients collectively through their representative organisations via the CHMP early contact with patient organisations (see page 27) and in stakeholder meetings in conjunction with the PRAC.

Scientific advice and protocol assistance

At any stage of a medicine's development, a developer can ask EMA for guidance on the best method for generating robust evidence of a medicine's benefits and risks. This is called scientific advice or, in the case of orphan medicines, protocol assistance. The added value and impact of patient input to scientific advice or protocol assistance procedures has been demonstrated, as this has helped to improve the advice provided.

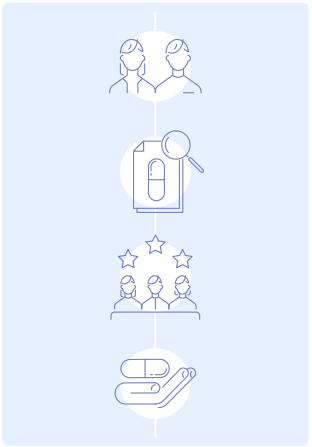
In 2022, 79 patients and carers participated in 79 scientific advice / protocol assistance procedures. In 2023, 43 patients and carers participated in 41 procedures.

Key achievement

The added value of patient engagement in EMA early dialogue at EMA and its impact on regulatory decision-making was demonstrated and documented in a peer-reviewed publication, using patient engagement in scientific advice as a case study.

Scientific advisory and ad hoc expert groups

Scientific advisory groups (SAG) or ad hoc expert groups (AHEG) are expert groups that are convened to answer questions raised during a medicine's assessment. SAGs are usually requested by the human medicines committee (CHMP), the safety committee (PRAC), or the committee for advanced therapies (CAT). In some disease areas, there are permanent SAGs; in those where there is no SAG, an ad hoc expert group can be organised.



In 2022, 21 patients and carers participated in 11 SAG or ad hoc expert group meetings, and in 2023, 31 patients and carers participated in 18 such meetings.

Scientific committee consultations

During the review of a medicine, EMA scientific committees may need to reach out to patients with experience of the condition being treated to obtain specific information. Several patients were individually consulted in 2022 and 2023 and invited to provide comments in person, in virtual meetings or in writing (see table 2, below)

Table 2: Scientific committee consultations

Year	Committee	No. of consultations	No. of patients consulted
2022	COMP	5	9
	CHMP	2	3
	PDCO	2	19
2023	COMP	4	4
	CHMP	5	8
	PDCO	1	5

** meetings and written consultations, including surveys*

Review of document destined for the public

Patients review documents destined for patients and the general public, primarily the package leaflet accompanying a medicine and the medicine overview published on EMA's website, prior to their publication. The suggestions and comments made by patients help to ensure that the documents address the target audience and use the right language to ensure the message is clear and understandable.

Patients reviewed 96 package leaflets and 54 medicine overviews in 2022 and 75 package leaflets and 45 medicine overviews in 2023. Patient also reviewed one safety communication in 2022 and five in 2023.

CHMP early contact with patient organisations

To address the need for engagement with patient organisations in the early stages of the evaluation of marketing authorisation applications (MAA), a pilot was conducted in 2021-2022 to involve patient organisations in procedures for orphan medicines. In this pilot, patients were invited to share their experiences and concerns about their condition and highlight key aspects that are important to them.

Feedback on the pilot was positive and it was considered that insights from patients are a useful complement to the assessment of the MAA dossier and the development of the CHMP initial assessment report. This engagement methodology was therefore adopted and extended beyond orphan medicines in 2022, and further expanded in 2023 to include healthcare professional organisations. Early contact is additional and complementary to the other engagement methodologies and targets organisations representing patients, consumers or healthcare professionals that fulfil EMA's eligibility criteria.

Key achievement

Following a successful pilot, the CHMP early contact with patient organisations was adopted as a core methodology and extended to all medicines, based on the positive experience with orphan medicines, adding to other existing methods for gathering patient input into medicines development and evaluation.

Patient organisations contributed to a training webinar for healthcare professional organisations in April 2023 in which they gave presentations on their experience with the CHMP early contact. A guidance document for patient and HCP organisations with frequently asked questions was published in November 2023.

In 2022 there were 26 early contact procedures with responses, and in 2023 40 procedures, with 32 responses from patient organisations.

Targeted engagement with healthcare professionals

"EMA engages with healthcare professionals as individual experts and through organisations representing a wide range of professions, including general doctors, specialists from many fields, nurses and pharmacists. They add value by bringing their experience of clinical practice and the specific knowledge, expertise and needs of their respective professional communities."
Rosa Giuliani, HCPWP Co-Chair

The perspectives of healthcare professionals such as practicing clinicians, specialised nurses and pharmacists are critical to discussions around the challenges with the development and real-world use of medicines and the impact of regulatory decisions on clinical practice.

For this reason, and complementary to the multi-stakeholder approach covered in Section 2, EMA has specific methodologies for engaging with healthcare professionals and their representative professional organisations and learned societies. These are further detailed in EMA's framework of interaction and aim to harness the widest expertise available to inform EMA decisions, raise awareness and understanding of EMA's work and ultimately reinforce confidence and trust in the EU regulatory system.

During the reporting period, we marked 10 years of successful operation of the HCPWP and confirmed the HCP Policy Officers Group (POG) as a valued complementary group to strengthen engagement of eligible healthcare professional organisations with EMA activities, in complement to the HCPWP. In addition, the CHMP early contact with patient organisations was extended to healthcare professional organisations.

Further to these key achievements in EMA's engagement with healthcare professionals, the Agency initiated a reflection to review the current framework of interaction with healthcare professionals and their organisations in order to incorporate learnings gained since its last revision in 2016 and address new challenges and opportunities. Looking ahead, EMA will be focusing on how best to engage with new generations of healthcare professionals by establishing more structured collaboration with healthcare professional organisations,

students' associations and academia. Attention will be also given to areas for engagement related with public health, clinical practice and information on medicines, including for example shortages, electronic product information, antimicrobial resistance, mis- and disinformation, and the European Vaccination Information Portal. We will continue to promote awareness of existing engagement methodologies and available regulatory tools supporting medicines development and evaluation and further consolidate healthcare professionals' involvement in the full spectrum of EMA's activities, including the implementation of new legislation.

Healthcare Professionals' Working Party (HCPWP)

The HCPWP is a formal structure within EMA, composed of representatives of eligible healthcare professional organisations as well as representatives of all EMA Human Scientific Committees. Together with the PCWP, they identify opportunities and challenges that may need special attention, particularly in the context of the respective frameworks.

As in previous reporting periods, the HCPWP continued its activity informed by its workplan. A joint PCWP/HCPWP workplan 2022-2025 was developed taking into account the 2019-2022 workplan, the EMAN strategy to 2025, the Regulatory Science Strategy and feedback collected in 2021 from the working party members, and adopted in June 2022.

Throughout 2022-23 several topics were discussed and progressed as highlighted in table 3 and meeting summaries and presentations were systematically published.

Table 3: HCPWP involvement during 2022 and 2023

PCWP/HCPWP shared areas of work

Medicines development and evaluation	• Advanced therapy medicinal products (ATMPs) • Activities linked to presence of N-nitrosamines in medicines for human use • Biosimilar interchangeability • Patient experience data (PED) • Scientific Committees and Working Parties • Facilitating innovation in regulatory science: research needs and multistakeholder collaboration • Generating clinical evidence for treatment and prevention options for Long-COVID and Post-acute sequelae condition (PASC)
Availability and accessibility of medicines	• HMA-EMA Task Force on the availability of authorised medicines • Reporting of shortages by organisations

PCWP/HCPWP shared areas of work

	• EU guidance on shortage prevention and communication to the public • Communication on medicines shortages • Preparedness activities • EU list of critical medicines • Repurposing • Restriction of the use of per- and polyfluorinated alkyl substances (PFAS) • Support to HTA regulation implementation
Clinical trials	• ACT EU • PCWP/HCPWP drafting group on the ICH E6 guidance on Good Clinical Practice • Decentralised clinical trials • ICH E21 Concept paper on inclusion of pregnant and breastfeeding individuals in clinical trials
Data analytics, digital tools and digital transformation	• HMA-EMA Joint Big Data Steering Group work plan • DARWIN EU® • Delivering on stakeholders' requirements for AI
Safety of medicines	• Update on pharmacovigilance and new initiatives for risk minimisation • Minimisation of opioid use disorder (OUD)-risk with opioid-containing medicinal products • Report on pharmacovigilance tasks from EU Member States and EMA - 2019-2022
Information on medicines	• Electronic product information (ePI) • Revision of QRD template for package leaflet improvement • Mis- and disinformation • Revamp of the Human Medicines Highlights newsletter • Use of EMA communications and communication campaigns • New EMA policy on multilingualism

PCWP/HCPWP shared areas of work

Public health focus areas	• COVID-19 updates on development and safety surveillance of vaccines and therapeutics • Lessons learnt from COVID-19 • Clinical trials in emergency situations • Monitoring of events and preparedness for public health emergencies/major events • Antimicrobial resistance
Building transparency and trust	• OPEN pilot • Perception survey • Stakeholder engagement report • Involvement of patients and healthcare professional representatives in various EMA groups • Social media strategy • Corporate website accessibility
Training and support	• ATMPs dedicated webinar • How regulatory and HTA activities interconnect

HCPWP specific work

Advances in clinical practice and clinical research	• Convey Family Medicine principles in Health care and in Pharmaceutical care (EFPC) • Recommendations for reducing bureaucracy in clinical trials (EHA) • How to reach consensus for integration of randomised and non-randomised studies of interventions (RWE) into next-generation health guidelines (EAACI)
Framework for engagement	• Streamlining eligibility self-certification • The importance of stakeholders' engagement to support implementation of the European Medicines Agencies Network Strategy

HCPWP specific work

	• Reflection on the need to review EMA's framework of engagement with healthcare professionals and their organisations
Outreach to clinical practitioners/researchers	• Health Policy Officers' Group

Election of new HCPWP co-chair

In September 2022, the HCPWP elected Rosa Giuliani of the European Society for Medical Oncology (ESMO) as new co-chair. This was the beginning of a three-year mandate, shared with the appointed EMA co-chair, Juan Garcia Burgos, Head of Public and Stakeholder Engagement.

Key achievement

2023 marked the 10th anniversary of the establishment of the HCPWP. In June 2023, the HCPWP plenary meeting gathered former co-chairs to engage with all members in a reflection of the past, present and future of this working party and set the foundation for the coming years.

Healthcare professionals' contribution to EMA work

EMA's Management Board includes one doctor representative and one veterinarian representative.

Healthcare professionals are members of the following EMA scientific committees:

- Paediatric Committee (PDCO) – 3 members and 3 alternates
- Committee for Advanced Therapies (CAT) – 2 members and 2 alternates
- Pharmacovigilance Risk Assessment Committee (PRAC) – 1 member and 1 alternate

Healthcare professionals were represented in several EMA working groups and task forces, including the Medicines Shortages Steering Group (MSSG), Medical Devices Shortages Steering Group (MDSSG), DARWIN EU Advisory Board, Big Data Steering Group (BDSG), Emergency Task Force (ETF), Drafting Group on Digital Support Tools to Risk Minimisation Measures, Advisory Group on Raw Data, QRD working subgroup, PRAC multistakeholder working group on digital Support Tools to Risk Minimisation Measures (RMM), and ACT EU Multistakeholder Advisory Group.

In December 2023, there were 40 eligible healthcare professionals' organisations, of which 22 were members of the HCPWP. A satisfaction survey conducted among all eligible

healthcare professional organisations in 2023 indicated a high level of satisfaction with the responding organisations' engagement with EMA and with their role as an eligible organisation.

These organisations are EMA's first port of call when seeking external healthcare professionals who can contribute to EMA activities. A total of 127 EMA-nominated healthcare professionals were registered in the expert management tool by December 2023 and are readily available to be involved in specific EMA activities.

The involvement of practicing clinicians, specialised nurses and pharmacists is critical when seeking input on the clinical benefit of candidate medicines, medicines currently in use and how they are used, as well as a real-life perspective of how regulatory decisions can be translated into clinical practice. These healthcare professionals can be involved at different times during the same evaluation procedure.

- At the start of assessment (since May 2023): EMA contacts healthcare professional organisation(s) covering the therapeutic area of the MAA. Generally, these organisations are part of EMA's established network of eligible EU healthcare professional organisations and European Reference Networks (ERNs). Through a written consultation based on a questionnaire, the organisations are asked to share their experience and concerns about the condition(s) and key aspects that are important for them so that this can be taken into account in a timely manner during the assessment process. Between May and November 2023, relevant organisations were contacted in the context of 31 procedures and a total of 36 responses from these organisations were collected and shared with the rapporteurs and applicants for transparency.
- During the assessment: if there are additional questions and/or major objections from EMA's scientific committees, healthcare professionals (namely doctors specialised in the therapeutic area of the MAA) are involved in benefit/risk evaluations whenever a SAG/ad-hoc expert group meeting is requested by a scientific committee.
- In the context of safety reviews: EMA's safety committee (PRAC) can request a targeted stakeholder meetings with patient and healthcare professional organisations' representatives. Examples are provided in the highlight box below.

Healthcare professionals are also involved in the final review of safety communications (known as Direct healthcare professional communications (DHPC)) for medicines under the centralised procedure.

Involvement of healthcare professional organisations in stakeholder meetings in the context of safety reviews

VALPROATE

- In 2023, EMA held two virtual stakeholder meetings on valproate-containing medicines involving patient and healthcare professional representatives. These medicines are used to treat epilepsy and bipolar disorder and, in some EU countries to prevent migraine.
- The first meeting, held on 1 February 2023, was organised following an Article 31 referral that led to new measures to avoid valproate exposure in pregnancy and focused on maternal exposure to valproate. The meeting aimed to better

understand the potential barriers and enablers for successful implementation of the valproate pregnancy prevention programme in clinical practice, to understand important issues outside the regulators' remit, and to ensure continuous collaboration to strengthen implementation of the risk minimisation measures.

- The second meeting was held on 16 November 2023 and focused on paternal exposure to valproate, following new data suggesting a potential risk of neurodevelopmental disorders in children conceived by fathers taking valproate medicines. The purpose of the meeting was to discuss the proposed risk minimisation measures for male patients so as to inform PRAC recommendations.

FLUOROQUINOLONES

- A virtual stakeholder meeting with healthcare professional organisations on fluoroquinolone antibiotics was held on 13 June 2023, following an Article 31 referral and the outcome of a drug utilisation study to evaluate the effectiveness of the risk minimisation measures resulting from the referral. The meeting aimed to align relevant guidelines with the outcome of the EU-wide review, raise awareness of the side effects, and discuss ways to foster closer collaboration and engagement with members of the organisations so as to increase awareness of this safety issue. The meeting further discussed antimicrobial resistance stewardship and prudent use of antibiotics. As a concrete follow-up action, the European Association of Urology is engaging with EMA to co-develop webinars and congress sessions in 2024 and 2025 to increase awareness among their infectious disease and primary care interest groups.

Key achievement

In 2023, following the successful implementation of the methodology for engaging with patient organisations at the start of a new MAA evaluation by the CHMP which was piloted in 2021-22, EMA extended the implementation of this methodology to healthcare professional organisations. A webinar was held in April 2023 to present the methodology to the HCP organisations and involvement started in May 2023. More details can be found in the process and FAQs.

Healthcare professional policy officers' group

The number of eligible HCP organisations has considerably outgrown the number of available HCPWP membership places. To ensure that all organisations are heard and have the possibility to interact with EMA on relevant topics concerning public health, the HCP Policy Officers' Group (POG) was established in 2021 to complement the HCPWP.

The HCP POG brings the policy officers of eligible organisations together and aims to:

- further support engagement and communication with EMA-eligible HCP organisations;
- establish a common forum for organisations to raise points with EMA in a coordinated and transparent manner – focusing on the remit of EMA activities;
- provide complementary EMA updates to HCPWP meetings.

The group has become a central point for sharing information and identifying common areas of interest in which organisations would like to jointly draft organisation-driven position papers that bring their perspectives into critical areas of regulatory discussion.

A one-year review of the HCP POG pilot was conducted in January 2022. Overall, the feedback was very positive and there was strong support to continue the group beyond the pilot, incorporating the learnings and experience gained. The group's activities were therefore continued in 2022 and 2023, with 6 meetings each year and following an agreed list of topics of interest. In 2022, topics presented or discussed included medical devices expert panels; ACT EU/ ICH GCP modernisation; CTIS update; and EMA's scientific publication strategy. In 2023, topics for discussion included the need to revise the framework of interaction with HCPs and their organisations; ENCePP; shortage-related activities; EMA activities supporting development and evaluation of medicines for children; and an EMA workshop on generating clinical evidence for treatment and prevention options for long COVID and post-acute sequelae condition. Specific points raised by organisations were systematically discussed during meetings, as well as recent/upcoming events and recent publications of interest or public consultations. Members also had the opportunity to present their organisation's activities and key areas of policy work.

Drafting groups

It was agreed in April 2022 to establish drafting groups for the development of opinion statements. The first four topics selected were shortages; considerations relating to frailty and older people in medicines' development and evaluation; surrogate endpoints and new ways of conducting clinical trials; and pharmacovigilance, with a focus on medication errors. The second topic led to the publication of a co-authored article between representatives of eligible healthcare professional organisations and regulators entitled "Inclusion of functional measures and frailty in the development and evaluation of medicines for older adults" in *Lancet Healthy Longevity* in November 2023. Work is in progress for the remaining topics.

2023 re-assessment

A survey was conducted at the end of 2023 to reassess the usefulness of the HCP POG and the satisfaction of its members, and to inform next steps. There were 32 responses out of 39 eligible organisations. The results were positive for all topics, including achievement of objectives, meetings, and drafting groups, with all responders seeing an added value in continuing the group. Suggestions will be taken on board to refine the group's scope and activities for 2024.

Key achievement

Confirmation of the HCP POG as a valued group to strengthen engagement of eligible healthcare professional organisations with EMA activities, complementing the HCPWP.

Bilateral meetings

Bilateral stakeholder meetings can be organised upon request by a healthcare professional organisation to discuss issues of common interest. Priority is given to EMA-eligible organisations as they have already been scrutinised against eligibility criteria endorsed by EMA's Management Board, which include transparency on the organisation's funding sources.

From EMA's perspective, these meetings are intended to listen and understand the stakeholder's areas of concern, provide additional information/clarifications where possible and, when relevant, identify areas where a multistakeholder approach may be more efficient. They complement discussions taken at the level of the HCPWP and PCWP, where it is not possible to go in depth into each organisation's more specific areas of concern or subject matters. Confidential and product-specific discussions are out of scope for such bilateral meetings.

Throughout the reporting period, bilateral meetings were organised with eight different healthcare professional organisations and a joint meeting also took place with the organisations representing general practitioners.

Key achievement

Greater awareness and use of this engagement methodology throughout 2022 and 2023 has facilitated better understanding of regulatory procedures among healthcare professional organisations and early identification of areas of concern and topics of common interest that can be further addressed, for example, in co-developed sessions in the context of the organisations' annual congresses. In 2024, EMA will continue to work towards transparency by publishing summaries of these meetings.

Contributions to healthcare professional organisations' meetings

The Agency regularly contributes to events such as congresses, workshops, and courses organised by eligible organisations. This is in line with the corresponding engagement framework and aims to raise awareness of the Agency's activities and encourage and facilitate the participation of healthcare professional organisations in the Agency's work.

Selected examples include:

- a dedicated session during the 16th Congress of the European Society of Contraception and Reproductive Health in May 2022, addressing EMA's relevant activities and how the Agency engages with healthcare professionals;
- a presentation on EMA's activities related to shortages during the European Association of Hospital Pharmacists (EAHP) annual congress in March 2023;
- a dedicated session during the European Alliance of Associations for Rheumatology (EULAR) annual congress in June 2023, covering a general introduction to EMA activities and the current status of medicines in the field of rheumatology;
- a presentation on EMA's relevant activities and how the Agency engages with academics and healthcare professionals as part of the Clinical Research Training in Hematology (CRTH) session organised during the European Hematology Association (EHA) annual congress in June 2023;
- contribution to a European Society of Cardiology (ESC) round table on unmet medical needs in November 2023, with participation of members of EMA's Cardiovascular Working Party (CVSWP).

Federation of Veterinarians of Europe

Veterinarians and other animal health practitioners play a critical role in maintaining the health and welfare of animals in the EU. The Agency strives to build a strong collaboration with the veterinary community and frequently holds joint events in partnership with the Federation of Veterinarians of Europe.

In 2022, the EMA focussed on supporting activities related to the implementation of the Veterinary Medicinal Products regulation.

In June 2023, EMA organised a workshop to collect insights on what information veterinary healthcare professionals would find beneficial in their daily practice. During the second half of 2023, the Agency held two focus group meetings on pharmacovigilance activities and reporting of adverse events in the veterinary field.

Main events:

- European Medicines Agency (EMA) and Federation of Veterinarians of Europe (FVE) webinar on the Union Product Database website
- Focus group on veterinary pharmacovigilance reporting in poultry
- Focus group on veterinary pharmacovigilance reporting in aquaculture

Targeted engagement with academia

" At the Agency, we firmly believe that strengthening the connection between Regulatory Science and Academia will expedite the delivery of transformative innovations for the benefits of all patients. "

Emmanuel Cormier, Head of Regulatory Science and Innovation

EMA is committed to maintaining a strong working relationship with the European academic sector, including public and non-for-profit organizations involved in research and development in the fields related to the work of the Agency. Such collaboration is necessary for the Agency to be prepared for future challenges as well as for leveraging opportunities offered by advances in science and technology. The Agency has a broad range of targeted engagement with academia, learned societies and research groups in a range of areas.

While there is often an overlap between academic stakeholders and healthcare professional stakeholders, the engagement reported herein relates to academia, including public and non-for-profit organizations, in its role for advancing knowledge to develop technologies and in particular regulatory science and medical technology.

The EMA has also progressed its internal Academia Collaboration Matrix action plan, which is aligned with the European Medicines Agencies Network Strategy and the Regulatory Science Strategy. The action plan guides EMA's engagement with academic stakeholders, which follows three main lines of activity (research, innovation and networking & training). An internal EMA group, the Academia Matrix, contributes diverse expertise and viewpoints to the action plan.

Engagement in research

It is important for the Agency to be aware of the latest scientific knowledge and its application on innovations and methodologies. The Agency actively supports research in relevant areas in order to pursue its mission to foster scientific excellence in the evaluation and supervision of medicines. EMA participates in external research projects in close collaboration with academic stakeholders to support regulatory science research and to

contribute to research outcomes aligned with or advancing regulatory standards, as well as to facilitate their translation to patients, healthcare professionals and health systems.

Such external research projects include those funded under Horizon Europe and by the Innovative Health Initiative (IHI). In particular, EMA scientific staff is involved in over 65 public-private partnerships for research in an early, pre-competitive stage such as Innovative Medicines initiative (IMI), IMI2, Horizon 2020 and Horizon Europe. These partnerships with academic/research centres undertake research in strategic areas such as regulatory science research needs. In the period 2022-2023, EMA research collaborations yielded over 30 peer-reviewed publications, helping to advance regulatory science and to translate it into regulatory practices.

The Agency also collaborates with research funding institutions such as the European Commission, research executive agencies and national funding organisations, to make recommendations for proposals and to provide regulatory and scientific input on specific elements in planned funding calls.

EMA further collaborates with networks of researchers on particular topics, such as the European Network of Paediatric Research at the European Medicines Agency (Enpr-EMA). This is a network of research networks, investigators and centres with recognised expertise in performing clinical studies in children. It acts as a platform for sharing good practices as well as a pan-European voice for promoting research into medicines for children. Information on Enpr-EMA annual meetings, newsletters and database of networks and organisations for paediatric research can be found on the dedicated webpage.

Engagement on innovation

The academic sector is a key originator of transformative medicines. To facilitate the transfer of these innovative solutions to industry, it is important that there is engagement with regulators at an early stage of development. Moreover, novel technological advances in biomedicine have made it easier for academics to develop solutions for unmet medical needs or for certain patient populations such as those affected by a rare disease or those that may not be financially interesting for industry. In such situations, sustained interaction with regulators can pave the way to patient access.

Incentivised regulatory support tools

EMA recognises that academia faces challenges when it comes to engaging with regulators. To address this, EMA facilitates engagement by offering specific interactions and incentivised programmes.

Academia briefings provide support tailored to non-for-profit researchers and developers. This interaction with EMA scientific staff at an early point of contact enables the exchange of experience, guidance on the regulatory system and discussions over development options. The interaction is informal and free of charge. The academia briefings are also an important opportunity for EMA to engage with the academic developers on regulatory challenges and research needs in concerned areas. In the period 2022-23, EMA held around 20 academia briefing meetings.

Key achievement

Academia Briefings have been established as a new regulatory tool tailored to the academic sector. In 2023 the EMA provided support to around 20 academic developers and researchers.

Academic developers may also request an Innovation Task Force (ITF) briefing meeting, as any other developer. These free-of-charge meetings are intended to facilitate the provision of guidance early in the development process and cover regulatory, technical and scientific issues arising from the development of innovative medicines, new technologies and borderline products.

Academic stakeholders are also offered incentives in the development of orphan medicinal products. For example, fees are waived for protocol assistance (scientific advice) when a product received OMP designation. This helps to ensure the product development is aligned with regulatory standards and may help the academic developer to transfer the product to industry for commercial development.

Further incentives are offered to academia through the PRIME scheme where, based on compelling non-clinical data and tolerability data from initial clinical trials, academic applicants can apply at an earlier stage than other applicants. The programme provides a full fee waiver for scientific advice and follow-up scientific advice for developing PRIME-designated products.

Academics developers of advanced therapy medicinal products (ATMPs) are offered earlier dedicated interaction and a 65% fee waiver for scientific advice.

EMA has also launched special pilot programmes to support academic developers on specific regulatory development pathways and products.

Repurposing pilot

Aimed at supporting the academic sector in generating sufficient evidence on the use of an established medicine in a new indication, with the view to having this new use authorised by a regulatory authority.

This approach can make new treatment options available to patients. During 2022-23, the repurposing pilot project supported academic champions with academia briefing meetings, pre-submission meetings for scientific advice and scientific advice, benefiting from a decision of the EMA Executive Director to provide scientific advice free of charge.

ATMP pilot for academia and not-for-profit organisations

In September 2022, EMA launched a pilot to support academic sponsors and not-for-profit organisations who are developing ATMPs. During the pilot, which aims to optimise ATMP development, EMA will provide enhanced regulatory support for up to five selected ATMPs that address unmet clinical needs and are being solely developed by academic and not-for-profit developers in Europe.

EMA will guide the pilot participants through the regulatory process, including offering advice on best practice principles for manufacturing and clinical development plan to required regulatory standards. On 8 February 2023, EMA hosted a webinar to introduce the pilot and offer the opportunity to potential applicants to raise questions. The selected pilot participants will benefit from fee reductions and waivers as stated in the Decision of the Agency's Executive Director on fee incentives for scientific advice, marketing authorisation applications and pre-authorisation inspections.

Key achievement

Pilot programmes launched offering tailored support to the academic sector on repurposing in 2022 and for the development of ATMPs in 2023.

Engagement in networking and training

EMA encourages academic stakeholders to register with EMA as either an organisation or an individual to indicate their interest in EMA activities. Since individual academic researchers and developers have expertise on a particular subject or area, registration helps EMA to identify and communicate relevant opportunities for participation or collaboration in an academic stakeholder's particular area of interest, as well as being able to highlight events such as workshops or opportunities to work with EMA scientific staff on aspects of regulatory science. A total of 50 individual academic researchers and developers registered with EMA during 2022-2023.

In the period 2022-23, EMA received a growing number of requests for information from academic stakeholders via the online enquiry form. Requests often concerned medicines, regulatory science topics, guidelines and the regulatory system. This included requests for educational visits to the Agency. This interest, together with EMA's goals for communication and transparency, were addressed by the Agency's Academia Info Day first held in November 2023, an in-person event that was also broadcast.The Info Day provided general information about EMA and its activities, as well as information on the regulatory support offered to the academic sector.

In February 2023, EMA held a regulatory and scientific conference on RNA-based medicines in which academic and industrial developers discussed the challenges of RNA-based innovative medicines, and scientific and regulatory opportunities as well as gaps in regulatory science. The virtual conference focused on emerging RNA technologies beyond vaccines and aimed to promote their development. Academic developers showcased technologies and applications in different therapeutic areas.

The EMA also collaborated with the European Infrastructure for Translational Medicine (EATRIS) on the ADVANCE EU training project for ATMP developers and held webinars to help ATMP developers navigate the regulatory framework for advanced therapies. Recorded webinars are available. EATRIS and EMA also created a video tutorial on how to apply for and benefit from an orphan drug designation.

EMA and EMRN pharmaceutical quality experts have formed the Quality and Innovation Group (QIG) which supports the translation of innovative approaches to the design,

manufacture and quality control of medicines for the benefit of patients. In 2023 the QIG launched a series of listen-and-learn focus group meetings with stakeholders from industry, academia and international regulators to hear about the regulatory challenges developers face in relation to innovative products, processes, control strategies and facilities, and to identify potential solutions. These are knowledge exchange and information events on highly innovative methodologies.

In 2023 EMA's newly established 3R Working Party ('Replace – Reduce – Refine' animal studies) held a public plenary meeting to showcase the Agency's commitment to the implementation of the 3Rs principle in the development and evaluation of human and veterinary medicinal products. This area is of high interest to academic researchers and developers.

Cancer Medicines Forum

In collaboration with the European Organisation for Research and Treatment of Cancer (EORTC), EMA launched the Cancer Medicines Forum (CMF) in March 2022. Bringing together representatives from academic organisations and the European medicines regulatory network, the forum aims to advance research into optimising cancer treatments and will help foster high standards in cancer care in the EU. Recent innovations in oncology such as personalised medicines, immunotherapies and ATMPs have helped cancer patients across Europe. However, at the time when new medicines enter the market, there is still ample opportunity for improvement, for example in terms of optimising their use and integration into the existing array of treatments. Aacademic organisations from EMA's Healthcare Professionals Working Party are included in the CMF discussions, which support the prioritisation of actions to improve the use of cancer medicines and delivering the Regulatory Science Strategy to 2025 and the Academia Collaboration Matrix Action Plan.

Participation of oncology scientists in medicine regulation (pilot project)

As part of the efforts to further advance collaboration with academia, EMA fosters the participation of academic experts in medicines regulation. Academic experts can be involved in the Agency's work as European experts in the context of the authorisation, supervision, and maintenance of medicinal products for human or veterinary use. In May 2023, EMA and the HMA launched a pilot project to enable clinical-oncology scientists to participate in medicine regulation. The pilot consists of live and recorded webinars covering basic principles of the regulation of human medicines in the EU and regulatory aspects relevant to oncology. It aims to increase collaboration between regulatory authorities and stakeholders in healthcare and academia.

Key achievement

Generation of training material, Info Days and discussion forums tailored to and in collaboration with the Academic Sector.

Targeted engagement with industy

EU trade industry associations (human and veterinary) are one of EMA's main stakeholder groups. Engagement with these key stakeholders aims to promote dialogue and understanding on common challenges, ensure transparency and timely dissemination of key information and foster a mutual understanding of each other's strategic priorities. This type of engagement takes place in parallel with core business interactions with single companies and promotes effective engagement, good organisational governance and accountability.

The areas of interaction with industry stakeholders are at a strategic and operational level and cover both human and veterinary fields.

Strategic level

Industry Standing Group

The Industry Standing Group (ISG) was set up in June 2022 to regularly exchange views, promote dialogue and receive feedback from industry stakeholders on issues of strategic, cross-industry common interest related to human medicines within the European legal framework, and facilitate implementation of new legislative proposals.

Initially the discussions focussed on the implementation of EMA's extended mandate (Regulation EU 2022/123). In this context, the ISG was used to update stakeholders on the activities addressing medicines shortages arising from the COVID-19 and Mpox public health emergencies. It was also used to foster continuous discussion and collaboration on the successful development of the European Shortages Monitoring Platform (ESMP) and the Critical Medical Devices Shortages (CMDS).

In addition, stakeholders had the opportunity to learn more about the activities of the HERA and JICF.

The ISG also discussed ETF initiatives to ensure expedited and coordinated medicines approval and the pilots linked to the Notified Body Conformity Assessment, including EMA coordination of experts opinions on the assessment for class II and class IIb medical devices

(CECP) and expert views on the performance evaluation report for class D in vitro diagnostic medical devices (PECP).

Towards the end of 2022, the scope of the ISG was extended to include cross-industry strategic topics for which there was no dedicated forum for discussion. In this context, several updates and clarifications were provided on the EMA Agile transformation initiative, the implementation of the CTR and aspects of CTIS, the ACT EU initiative, and on the restructuring of EMA working parties. Another topic discussed at the ISG is the proposed reform of the EU pharmaceutical legislation; ISG will serve as the main platform for engagement with industry for the upcoming revision of this legislation.

The ISG enables strategic discussions on key priorities and complements existing forums, such as industry stakeholder platforms, where more operational aspects are addressed, such as those linked to medicines development support, the centralised procedure and pharmacovigilance.

Key achievement

Following a one year pilot, the ISG was confirmed as a group to engage on the implementation of EMA extended mandate and other areas of strategic interest.

Bilateral interactions

In 2022 and 2023, EMA organised a number of bilateral meetings with several eligible Industry EU trade organisations to enable industry stakeholders to discuss topics of particular interest and raise awareness on challenges experienced by a specific sector of the industry or trade association.

In addition, as part of EMA's internal training activities, representatives from two industry trade associations joined the H-Division meeting in October 2023 to present industry perspective on Good Manufacturing (GMP) and a Good Clinical Practices (GCP) regulatory inspections.

The following bilateral meetings took place in 2022 and 2023 with minutes and agendas published on the EMA corporate webpage (see annex "Regular stakeholder meetings with industry representation held in 2022-2023 " for the complete overview).

Eligible organisations	Key topics discussed
Association of the European Self-Medication Industry (AESGP)	Implementation of medical device regulation, ePI.
Affordable Medicines	Metrics of parallel distribution submissions, requirements of the UPD, regulatory check of parallel distribution.

Eligible organisations	Key topics discussed
The European Federation of Pharmaceutical Industries and Associations (EFPIA)	COVID-19 lessons learnt, implementation of the HTA regulation and in vitro diagnostic regulation, development of the EMSP.
European Confederation of Pharmaceutical Entrepreneurs (EUCOPE)	EUCOPE's regulatory vision and 2025 priorities, CTIS implementation, use of data sources for ATMPs and oncology medicines, reform of the EU pharmaceutical legislation.
EuropaBio	Europe's pharmaceutical strategy and the future of biotechnology, use of real world evidence in decision making, the ACT EU initiative, implementation of the CTR, international regulatory convergence.
Medicines for Europe	Off-patent medicines, biosimilar medicines access and availability, and manufacturing and supply chain oversight.
MedTech Europe	Reform of the EU pharmaceutical legislation and activities linked to medical devices and in vitro diagnostics.
Nuclear Medicines Europe	Common Technical Document (CTD) regulatory requirements for the radionuclide and chemical precursor, use and availability of radioligand therapeutics and radiopharmaceutical imaging agents.
Vaccines Europe	Learnings on the regulation of vaccines in the EU, international cooperation, the impact of EMA's extended mandate on vaccines, cooperation between EMA, HERA, and ECDC, vaccines hesitancy.

Smart regulation

The increasing use of digital tools and AI to promote advances in technology and smart regulation was another important topic for engagement with industry EU trade organisations. The discussions focused on EMA's transition into the Scaled Agile Framework (SAFe) and on the use of new technologies in the pharmaceutical environment.

- Since 2021, EMA has transformed its approach to information technology (IT) by embracing the SAFe mindset which has been implemented in EMA's governance, delivery and operations, based on a five-year roadmap due to be completed in 2027. The network portfolio is now organised around five value streams:

    1. Research and development (R&D) – to support the development of new medicines and generation of scientific evidence.
    2. Product lifecycle management (PLM) – to manage the authorisation and lifecycle of medicines and certain medical devices.

3. Monitoring – to monitor the availability and safety of products.
4. Managing the Agency – to manage EMA, and coordinate and support the European medicines regulatory network.
5. Technology lifecycle management and information security – to manage information technology and security.

- In this context, industry EU trade organisations had the opportunity to provide strategic input through their participation in Quarterly Strategic Portfolio Review ceremonies. Industry stakeholders were additionally involved at development level through 'industry subject matter experts' nominated by industry EU trade organisations (see Section 6.2.2.8).
- Discussions on the use of AI and other advanced technologies in medicines regulation were continued via the joint HMA-EMA Big Data Steering Group. In July 2023 this group published a reflection paper on the use of artificial intelligence in the lifecycle of medicines for public consultation. In addition, dedicated events were organised to foster discussions on the potential use and impact of such technologies in the near future of medicines regulation.

Veterinary info days

Each year EMA holds events to provide veterinary industry stakeholders with updates on veterinary regulatory policy, scientific and procedural developments and to collect feedback from these stakeholders. During 2022-2023, key topics discussed were largely related to implementation of Regulation (EU) 2019/6 on Veterinary Medicinal Products, namely pharmacovigilance, GMP revision and innovation.

Eligible organisations	Key topics discussed
AnimalhealthEurope, AccessVetMed and **AVC**	In the margins of the Veterinary Info Days: Veterinary Medicinal Products regulation, UPD, UPhD.

Operational level

During 2022 and 2023, discussions also took place on some of the technical and scientific aspects linked to the development, authorisation, managing and monitoring of medicines. Such meetings were held in the context of existing groups and forums and enabled a closer collaboration between industry stakeholders and EMA in the areas of evidence generation, medicine evaluation and monitoring. The main forums were:

- Industry stakeholder platforms
- Interested parties meetings with committees and domains
- Topic specific discussions (CTR and CTIS, SAFe transformation, EU big data workshops)
- Targeted engagement with SMEs.

Industry stakeholder platforms

There are currently 3 platforms covering Research and Development (R&D), Centralised Procedures (CP) and Pharmacovigilance (PhV). Each platform meets industry interested parties and provides the opportunity for regular engagement between regulators and representatives of industry stakeholder organisations in order to discuss matters linked to R&D, CP and PhV.

- Between 2022 and 2023 the **Industry Stakeholder platform on R&D** met four times, addressing key areas linked to evidence generation and development support. This included discussions on the activities aiming to strengthen frameworks for engagement on evidence planning, enhance the PRIME scheme, optimise support to paediatric developments, as well as foster the assessment of orphan maintenance applications. To enable more in-depth discussions, three focus groups were set up and reported back to the platform:
  - Focus group on review and strengthening the framework for qualification of novel methodologies (QoNM)
  - Focus group on the practical application of principles relevant for the Paediatric Investigation Plans (PIP) framework
  - Focus group on provision of scientific advice for medicinal product developments comprising drug-device combinations and drug-companion diagnostic combinations.

Other highlights from the R&D stakeholder platforms include an exchange with industry stakeholders on scientific advice capacity and related optimisation of guidance, progressing parallel joint scientific consultations (including the launch of the parallel EMA/HTA body (HTAb) scientific advice from September 2023), the implementation of the recommendations from the PRIME 5-year review, providing updates on advice to medical device manufacturers from the expert panels, as well as initiatives to strengthen patient-centric development.

- **Industry stakeholder platform on the operation of the centralised procedure** met also four times between 2022 and 2023. Key aspects discussed were the development of the IRIS platform, implementation of Medical Device Regulation, working parties governance model, restart of publication of clinical trials data in accordance with policy 0070, and initiatives such as the QIG, ePI and raw data pilots. Specific topics required additional discussion in focus groups with regular reporting to the platform:
  - CHMP Assessment Report Revamp Project
  - Focus group on submission predictability
  - Focus group on reliance.
- Other highlights from the stakeholder platforms on the centralised procedure include patient engagement in the development and authorisation of medicines, the integration of patient experience data in regulatory assessments, new initiatives such as the publication of RMPs, and changes to pre-submission meetings.
- The meetings of the industry stakeholders platform on PhV occurred once a year with the main goal of raising awareness of the requirements of the pharmacovigilance legislation and promoting the exchange of ideas, concerns and opinions. In this context, focus was given to PRAC Strategy on Measuring the Impact of Pharmacovigilance Activities,

updates to the Good Pharmacovigilance Practices (EU-GVP), and the risk management plan transparency initiative.

Interested parties meetings with committees and domains

Interested parties meetings are an opportunity for industry stakeholders to engage on a more technical level with members of committees, working parties, working groups and procedure-specific groups. The frequency of such meetings reduced significantly during the recent public health emergencies. However, following the restructuring of EMA's working parties into 5 main domains (quality, non-clinical, methodology, clinical, veterinary) meetings with interested parties resumed in a more structured way during 2022 and 2023. These meetings provided industry stakeholders with the opportunity to not only engage in scientific discussions on various aspects relating to the authorisation of medicines, but also to provide input on the workplan of each group.

COMMITTEES

- Committee for Veterinary Medicinal Products (CVMP)

In 2023, the CVMP resumed its interested parties meetings, topics included availability of medicines, responsible use of antimicrobials, adoption of the 3R approach to reduce animal testing, pharmacovigilance and innovation.

- Co-ordination Group for Mutual Recognition and Decentralised procedures – Veterinary (CMDv)

During 2022 and 2023, CMDv hosted several meetings with interested parties to enable discussion on the veterinary industry experience and how to overcome challenges on important topics such as variations and the quality review documents (QRD) v9 templates implementation.

- Co-ordination Group for Mutual Recognition and Decentralised procedures - Human (CMDh)

In 2022 and 2023, the CMDh held meetings with interested parties twice a year to engage on topics include nitrosamine impurities, resources, medicines shortages, telematic projects (including ePI) and procedural optimisation. In line with the CMDh Multi-Annual Workplan, topic-focused meetings were organised, such as the one that took place in May 2023 to exchange views on a future revision of the Variation Regulation.

- Committee for Advanced Therapies (CAT)

In May 2023, the CAT hosted a hybrid stakeholder meeting to discuss certain important topics, including ATMPs comprising genetically modified organisms; the European Commission proposal for a centralised GMP/Environmental Risk Assessment (ERA) evaluation; the predictability of marketing authorisation applications; stakeholders feedback from the public consultation on the guideline in quality, non-clinical and clinical requirements for investigational ATMPs in clinical trial; use of registries; recent experience and learnings from an EMA/European Network for Health Technology Assessment (EUnetHTA) bilateral on ATMPs; and an exchange of views on platform approaches.

QUALITY DOMAIN

This domain includes all activities of the Quality Working Party (QWP), Biologics Working Party (BWP), Biosimilar Medicinal Product Working Party (BMPWP) and related wording/drafting groups (Quality Innovation Group (QIG), Operational Expert Groups (OEG), Formulation Working Group (FWG)).

- The QWP published its workplan and hosted its annual meetings in 2022 and 2023 with interested parties. These meetings enabled scientific discussions on innovation and sustainability in manufacturing and quality and the impact of new guidance and/or requirements for medicines, with further dedicated engagement where needed. The main areas for discussion were new requirements for polyfluoroalkyl substances (PFAS), titanium dioxide and nitrosamines impurities; the Packaging & Packaging Waste Regulation; quality requirements for synthetic peptides; near infrared spectroscopy (NIRS) and industry's experience with the PRIME toolbox.
- The QIG was established in 2022 by the EU regulatory network to support innovative approaches for the development, manufacture, and quality control of medicines for the benefit of patients in the EU. The QIG holds 'listen & learn focus group' meetings to gather information on the latest innovations. Such meetings bring together representatives from academia, industry, the regulatory network and QIG to share knowledge and experience, identify potential barriers in the regulatory framework that prevent implementation of technologies and guide towards solutions.
- The BWP published its workplan and hosted its annual meetings with interested parties, with discussions on various topics such as biological modelling approaches and manufacture; analytical technologies including multi-attribute methods; risk-based approach to process validation; adaptive control strategies; approaches for patient-centric specifications; and regulatory flexibilities to achieve climate, environment and sustainability goals.

NON-CLINICAL DOMAIN

- This domain includes the Non-clinical Working Party (NcWP), the 3Rs Working Party (3RsWP) and the Nitrosamines Safety Operational Expert Group (NSOEG).
- The NcWP published its workplan and also resumed its annual meetings with interested parties to discuss important topics linked to medicines safety, such as the identification of replacement excipients for titanium dioxide, 'one substance – one assessment', and acceptable intake for nitrosamines present in medicines. Important discussions were also held on reducing the use of non-human primates in drug development and the revision of the ERA guideline, and lessons learnt from the implementation of the ICH S11 guideline on nonclinical safety testing in support of development of paediatric pharmaceuticals.
- The 3RsWP published its workplan and had its first interested parties meeting in February 2023, which included an introductory public broadcast on the workplan and priorities for 2023, with an opportunity for stakeholders and the public to interact and provide feedback. Additional targeted sessions were also held to allow more focused discussions with pharmaceutical industry, contract research organisations (CROs), EU agencies, animal welfare organisations and research consortia.
- The Nitrosamines Implementation Oversight Group (NIOG): the risk-mitigating measures for N-nitrosamines, in line with the Article 5 (3) scientific opinion, and the related "call

for review" have continued throughout 2022 and 2023 with dedicated strategic discussions with interested parties in addition to scientific discussions at the level of the NSOEG and QWP. Industry stakeholders were kept up to date on guidance developments, including the establishment of new approaches to streamline the adoption of acceptable intake limits and provide advice on corrective actions.

METHODOLOGY DOMAIN

In October 2023, the Methodology Working Party (MWP) launched a public consultation on its revised 2022-2024 workplan to better shape the roadmap on the basis of evolving priorities. The workplan covers strategic and tactical topics, such as guidance on bioequivalence, pharmacokinetic data, modelling and simulation, clinical trials modernisation, real world evidence, data science and artificial intelligence. Stakeholders were given the opportunity to provide additional feedback through a stakeholder meeting in December 2023. The feedback received will be consolidated and revised as part of MWP interested parties' meeting planned for 2024.

CLINICAL DOMAIN

This domain includes therapeutic area-specific working parties, with related 3-year workplans: Central Nervous System Working Party (CNSWP); Cardiovascular Working Party (CVSWP); Oncology Working Party (OncWP); Rheumatology/Immunology Working Party (RIWP); Infectious Disease Working Party (IDWP); Vaccines Working Party (VWP); and Haematology Working Party (HAEMWP, previously the Blood Products Working Party (BPWP)).

In 2022 the annual meeting between EMA, International Plasma and Fractionation Association (IPFA) and Plasma Protein Therapeutics Association (PPTA) provided the opportunity to discuss key topics linked to clinical as well as to non clinical aspects of blood, plasma and tissue-derived medicines such as the EU Blood Cells and Tissue Directives, recognition of GMP inspections, implication of the EMA extended mandate and the need for topic-specific guidance and priorities.

VETERINARY DOMAIN

The annual Pharmacovigilance Working Party-Veterinary (PhVWP-V) Interested Parties meetings were held in September 2022 and 2023 and were a valuable opportunity to bring together industry stakeholders (Access VetMed, AnimalHealthEurope, Association of Veterinary Consultants), the Federation of Veterinarians of Europe and the PhVWP-V stakeholders to discuss on experience and challenges linked to the implementation of the Veterinary Good Pharmacovigilance Practices (VGVP).

OTHER GROUPS

- **Good Manufacturing and Distribution Practices Inspectors Working Group (GMDP IWG)**

The GMDP IWG held its annual meetings with interested parties in 2022 and 2023. Topics included updates on the progress of relevant GMP guidelines, regulatory flexibilities linked to ensuring supply chain performance in the context of COVID-19 and specific inspection-related topics. Another important topic was an update on the integration of the EudraGMDP

database with EMA's Organisation Management Service (OMS), including SPOR, and linked to the implementation of the new veterinary directive. In this regard, several training events were also held to familiarise industry stakeholders with the changes.

- **Good Clinical Practices Inspectors Working Group (GCP IWG)**'s theme of the 2023 annual meeting with interested parties was "Rethinking clinical trials". The aim of the meeting was to allow participants to exchange views and experience on the use of computerised systems in the conduct of clinical trials, for example in relation to the decommissioning of databases, audit trail and audit trail review, the distributed trial master file and direct remote access to identifiable personal and health data required in clinical trials.
- The **Quality Review of Documents Working Group (QRD Group)** met with industry representatives on four occasions between 2022 and 2023. The meetings focused on labelling-related topics and aimed to gain industry stakeholders' views on the adoption and implementation of QRD decisions/guidance and how this might impact their processes.
- The **Name Review Group (NRG)** was established by the CHMP to perform reviews of the (invented) names of medicinal products being assessed by the Agency. In November 2023, the NRG organised a joint teleconference with interested parties to present the main changes to the Guideline on the acceptability of names for human medicinal products processed through the centralised procedure (EMA/CHMP/287710/2014), following a thorough review of all external comments received during the EMA public consultation. Further clarifications were also provided on the most controversial topics, such as promotional messages, pronunciation difficulties, similarity with international non-proprietary names (INN) and umbrella branding.

Key achievement

Following closure of COVID-19 pandemic business continuity plan regular engagement with Industry stakeholders through annual bilaterals, platforms and dedicated Interested Parties meetings resumed in 2023.

Topic-specific discussions

OPERATIONAL DISCUSSIONS ON CLINICAL TRIALS REGULATION AND CLINICAL TRIALS INFORMATION SYSTEM

With the Clinical Trials Regulation (CTR) entry into application in January 2022, EMA organised several activities to support industry stakeholders in ensuring timely and effective implementation of both the CTR and CTIS. These included training and information events, webinars and workshops, in addition to stakeholder surveys and public consultations.

In July 2022, industry stakeholders were further consulted on how to ensure protection of personal data and commercially confidential information in documents uploaded to CTIS. Following the feedback received in writing and via the multi-stakeholder workshop, the final Guidance document on protection of personal data and commercially confidential information

(CCI) in CTIS and accompanying annexes were published in July 2023 and the Revised CTIS Transparency Rules in October 2023.

RESUMPTION OF CLINICAL DATA PUBLICATION FOR ALL MEDICINES

In 2023, EMA resumed publication of clinical data beyond COVID-19 medicines in a phased approach in accordance with Policy 00070. This activity was paused whilst EMA was in a business continuity setting. Industry stakeholders were briefed during a dedicated webinar in May 2023.

Key achievement

Publication of clinical data publication for medicines for new active substances restarted in September 2023.

EU BIG DATA STEERING GROUP MEETINGS WITH INDUSTRY STAKEHOLDERS

In 2022 and 2023 industry stakeholders representing both human and veterinary medicines had the opportunity to further interact with the HMA/EMA Big Data steering group and share their views on big data activities.

OPERATIONAL ENGAGEMENT ON SAFE TRANSFORMATION

As already outlined, 2022 and 2023 were very important years for industry stakeholders' engagement on the Agile transformation initiative which was launched in 2021. The Agency's implementation of the SAFe methodology to improve the software product development process and structure has since been completed and all projects were transitioned to the Agile governance in the first half of 2023.

Industry stakeholders had the opportunity not only to follow the several webinars, quarterly system demos and quarterly strategic review meetings in 2022-23, but also to directly contribute as subject-matter experts with their technical expertise to the development of several Agency's IT systems such as the Critical Medical Devices Shortages (CMDS), ESMP, ePI, Veterinary UPD, Electronic Application Form (eAF). The protocol for subject-matter experts and product owners was launched in 2022 and updated in 2023 to incorporate feedback and included impacted partners and stakeholders (at this stage, national competent authorities and industry stakeholders), to directly contribute to IT system solution development and delivery.

Engagement with micro, small and medium-sized enterprises

Mirco, small and medium-sized enterprises (SMEs) are recognised sources of innovation in the pharmaceutical sector. During 2022 and 2023, the Agency promoted innovation and the development of human and veterinary medicines by SMEs through dedicated financial and administrative assistance facilitated by the SME office.

Key industry-focused topics for SMEs included training, education and onboarding for CTIS, the veterinary legislation and the impact of the reform of EU pharmaceutical legislation for SMEs.

An overview of the support available to SMEs and 2022 annual report is published on EMA's website.

Conclusion and next steps

This report provides an overview of activities during 2022 and 2023 that were intended to foster engagement with EMA's key stakeholder groups, namely patients/consumers, healthcare professionals, academia, and industry EU trade organisations including SMEs.

The engagement strategy adopted for the period analysed took on board the learnings from recent public health emergencies and dialogue at multi-stakeholder level was pursued where possible, as well as targeted engagement with representative groups where warranted.

In the years to come, while ensuring that patients remain at the heart of the work done, it is key that such multi-faceted dialogue continues in order to enable mutual understanding, collaboration and knowledge-sharing, not only on crisis preparedness but also on scientific and technological developments.

Key activities with patient and consumer organisations going forward will include adapting the public assessment report template (EPAR) to reflect patient and healthcare professional input, such as that received via the CHMP early contact methodology. Opportunities for including patient input earlier will be explored concurrently with the patient experience data initiative, particularly with regard to evaluation of benefits and risks. Engagement on information and communication around medicines and vaccines will be strengthened, in particular on antimicrobial resistance and in tackling mis- and disinformation. As training remains critical to building the capacity of patient representatives and organisations to participate in EMA's work, there will be a mapping of training needs, with a focus on emerging complex topics such as big data, real-world data and artificial intelligence.

We will be focusing on how best to engage with new generations of healthcare professionals by establishing more structured collaboration with healthcare professional organisations, student associations and academia. Attention will be also given to areas for engagement related to public health, clinical practice and information on medicines, including for example shortages, ePI, antimicrobial resistance, mis- and disinformation, and the European Vaccines Information Portal. We will continue to promote awareness of existing engagement methodologies and available regulatory tools supporting medicines development and evaluation and further consolidate healthcare professionals' involvement in the full spectrum of EMA's activities, including the implementation of new legislation.

Regarding engagement with academia, EMA intends to further cooperate with this important stakeholder group to provide the relevant regulatory support and identify research needs and academic pipelines which represent the seeds of innovation.

The targeted engagement with EU industry trade organisations at strategic and operational level will be further promoted for both human and veterinary medicines, especially in the context of important topics where early input and dialogue are essential for the operation of the centralised procedure, monitoring and supervision of human and animal medicines, and advances in technology and innovation. In this regard, EMA's industry platform and bilateral meetings will continue to foster dialogue and opporunities for improvement based on experience. In addition, the ISG will continue to provide a forum for engagement not only on EMA's extended mandate implementation but also on future activities, such as the upcoming reform of the EU pharmaceutical legislation, and other relevant strategic initiatives.

Annexes

Satisfaction survey with patient, consumer and healthcare professional organisations

In the past, EMA stakeholder satisfaction surveys have been sent to individual experts. Recognising the need to map the evolving perceptions and needs of the organisations representing these important stakeholder groups, the 2023 satisfaction survey was sent to eligible organisations. The aim is to assess the organisations' satisfaction, gather feedback on the interactions, and ultimately improve EMA's engagement activities. The survey ran during February-March 2024 and was sent to all 42 eligible organisations representing patients and consumers and healthcare professionals. The response rate of eligible healthcare professional organisations was 45% and 21% for eligible patient and consumer organisations.

From healthcare professional organisations polled, 88% said they were satisfied or very satisfied with their interactions with EMA. Seventy-two percent said they thought being an eligible organisation was very valuable, and 28% said they found it valuable – a slight increase since the last survey done in 2021.

From patient and consumer organisations polled, 100% said they were satisfied or very satisfied with their interactions with EMA. Seventy-eight percent said they thought being an eligible organisation was very valuable, and 22% said they found it valuable. The results for patients and consumer organisations cannot be compared to previous surveys as they had been targeted at individuals.

It could be seen that the level of satisfaction was generally high, and positive comments were received, thought the low participation rate on the patient and consumer side means the result cannot be regarded as conclusive. Engaging with EMA appears to be held in high value by the organisations, and their expressed topics of interest and engagement methodologies are reflective of the Agency's priorities and ongoing work. The feedback received will feed into ongoing discussions on how to further improve EMA's engagement with its stakeholders.

Regular stakeholder meetings with industry representation held in 2022-2023

Event name	Topic	Participants	Frequency
EMA Veterinary Awareness Day	Regulatory	Multi-stakeholder	12-13/09/23
Clinical Trials Information System (CTIS): Walk-in clinic	CTIS	Multi-stakeholders	28/03/2022, 04/04/2022, 22/04/2022, 05/05/2022, 19/05/2022, 02/06/2022, 15/06/2022, 31/08/2022, 20/09/2022, 05/10/2022, 15/11/2022, 18/01/2023, 16/03/2023, 19/04/2023, 17/05/2023, 14/06/2023, 19/07/2023, 20/09/2023, 15/11/2023, 13/12/2023
Clinical Trials Information System (CTIS) bitesize talks	CTIS	Multi-stakeholders	Clinical Trials Information System (CTIS) bitesize talk: Requests for information (28/04/2022) Clinical Trials Information System (CTIS) bitesize talk: Modifications (31/05/2022) Clinical Trials Information System (CTIS) bitesize talk: Transitional trials and additional Member State concerned (MSC) application

Event name	Topic	Participants	Frequency
			(23/06/2022) Clinical Trials Information System (CTIS) bitesize talk: Deferral rules and Public website (20/07/2022) Clinical Trials Information System (CTIS) bitesize talk: Notifications - Part 1 (28/09/2022) Clinical Trials Information System (CTIS) bitesize talk: Notifications - Part 2 (23/11/2022) Clinical Trials Information System (CTIS) bitesize talk: Annual safety report (ASR)(15/12/2022) Clinical Trials Information System (CTIS) bitesize talk: Initial clinical trial application (23/03/2022) Clinical Trials Information System (CTIS) bitesize talk: User access and role management (24/02/2022) Clinical Trials Information System (CTIS) bitesize talk: Document and

Event name	Topic	Participants	Frequency
			personal data in CTIS (23/02/2023) Clinical Trials Information System (CTIS) bitesize talk: IMPD-Q only submission (10/05/2023) Clinical Trials Information System (CTIS) bitesize talk: How to submit a transitional trial in CTIS (21/06/2023) Clinical Trials Information System (CTIS) bitesize talk: Part I-only applications and Part II requirements in CTIS (30/08/2023) Clinical Trials Information System (CTIS) bitesize talk: Training materials, CTIS pre-requisites, and updates on transparency rules (29/11/2023)
Clinical Trials Information System (CTIS) sponsor end user training programme	CTIS	Multi-stakeholders	24-27/01/2022 , 15-18/02/2022 , 04/03/2022 , 05-08/04/2022, 10-13/05/2022, 20-23/06/2022, 20-23/09/2022, 07-10/11/2022, 07-10/02/2023,

Event name	Topic	Participants	Frequency
			02-05/05/2023, 27-30/06/2023, 19-22/09/2023, 10-13/10/2023, 11-14/12/2023
Organisation Management System (OMS) Trouble Shooting Session for CTIS users	CTIS	Multi-stakeholders	30/06/2022, 27/07/2022, 22/09/2022, 19/10/2022, 24/11/2022,
Clinical Trials Information System (CTIS) info days and webinar	CTIS	Multi-stakeholders	Clinical Trials Information System (CTIS) demonstration for stakeholders (20/01/2022) Clinical Trials Information System (CTIS) Webinar - 9 months on and going forward - November 2022 (16/11/2022) Clinical Trials Information System (CTIS): Readiness for mandatory use of the Clinical Trials Regulation from 31 January 2023 (20/01/2023) Clinical Trials Information System Webinar: Second Year of Transition (04/07/2023) Clinical Trials Information System (CTIS): Information day (17/10/2023)

Event name	Topic	Participants	Frequency
HMA/EMA Big Data Stakeholder Forum 2023	Big data	Multi-stakeholder	04/12/23
HMA/EMA Big Data Stakeholder Forum 2022	Big data	Multi-stakeholder	01/12/22
Third Veterinary Big Data Stakeholder Forum	Big data	Multi-stakeholder	23/11/23
Second Veterinary Big Data stakeholder forum	Big data	Multi-stakeholder	23/11/22
Industry Standing Group	Policy/Strategy	Individual Human Industry Stakeholder Associations, EMA, Committee and Member States Regulators as appropriate	ISG 21/06/22 ISG 26/06/22 ISG 21/11/22 ISG 21/03/23 ISG 26/06/23 ISG 21/09/23 ISG 23/11/23
Bilateral Meetings with key Industry Associations	Policy/Strategy	Individual Industry Stakeholder Associations, EMA, Committee and Member States Regulators as appropriate	AESGP 14/02/2022, 18/04/23 EFPIA 21/03/22, 07/02/23 EuropaBio 07/06/22, 06/07/23 Medicines for Europe 15/09/22, 06/12/23 EUCOPE 11/10/22, 29/11/23 Nuclear Medicines Europe 11/10/22, 15/06/23 Affordable medicines 16/11/22

Event name	Topic	Participants	Frequency
			MedTech Europe 11/04/23 EuropaBio 06/07/23 Vaccines Europe 28/11/22, 27/11/23
Industry Platform meeting on research and development support	Research and Development	Industry Stakeholder Associations, EMA, Committee and Member States Regulators as appropriate	R&D platform 11/07/22 R&D platform 05/12/22 R&D platform 11/07/23 R&D platform 05/12/23
Industry Platform meeting on the operation of EU Pharmacovigilance legislation	Pharmacovigilance	Industry Stakeholder Associations, EMA, Committee and Member States Regulators as appropriate	PhV platform 07/11/22 PhV platform 22/11/23
Industry Platform on the operation of the centralised procedure for human medicines	Centralised procedure	Industry Stakeholder Associations, EMA, Committee and Member States Regulators as appropriate	CP platform 27/06/22 CP platform 24/11/22 CP platform 27/06/23 CP platform 24/11/23
Veterinary info days	Regulatory	"Veterinary" Industry stakeholders	12/05/2022-13/05/2022 16/02/2023-17/02/2023
SAFe Quarterly Strategic Portfolio Review	SAFe strategic portfolio	Industry stakeholders	14/06/2022 29/11/2022 25/04/2023 06/09/2023
CVMP Interested Parties' meeting	Regulatory	Industry stakeholders	24/05/23

Event name	Topic	Participants	Frequency
CMDh Interested Parties' meeting	Regulatory	Industry stakeholders	15/11/23 31/05/23 16/05/23 09/11/22 18/05/22
CMDv Interested Parties' meeting	Regulatory/Veterinary domain	Industry stakeholders	16/06/23 24/03/23 20/01/23 07/10/22 17/06/22 18/03/22 21/01/22
PhVWP-V Interested Parties	Pharmacovigilance	Industry stakeholders	27/09/2023, 28/09/2022
Committee for Advanced Therapies (CAT) meeting with interested parties	Regulatory	Industry stakeholders	16/05/23
Quality Working Party IP	Quality	Industry stakeholders	03/05/2022 27/06/2023
Biologics Working Party IP	Biologics	Industry stakeholders	12/05/2022 06/09/2023
Listen-and-learn focus group meeting of the Quality Innovation Group	Innovation	Industry stakeholders	17/05/23
Second listen-and-learn focus group meeting of the Quality Innovation Group	Innovation	Industry stakeholders	12-13/10/23
Non clinical working party	Non clinical domain	Industry stakeholders	03/10/2023
3Rs Working Party (3RsWP) plenary meeting - Public	Non clinical domain	Industry stakeholders	28/03/23

Event name	Topic	Participants	Frequency
session on the 2023 work plan			
Nitrosamines Implementation Oversight Group (NIOG) meeting with Pharmaceutical industry	Non clinical domain	Industry stakeholders	04/05/22 30/11/22 12/07/23 07/12/23
Methodology Working Party stakeholder interaction meeting	Methodology domain	Industry stakeholders	07/12/23
Good Manufacturing and Distribution Practices Inspectors Working Group meeting with Interested parties	Inspections, Good manufacturing practices, good distribution practices	Industry Stakeholders	10/03/2022 03/02/2023
Good Clinical Practices Inspectors Working Group meeting with Interested parties	Inspections, Good clinical practices	Industry Stakeholders	18/06/2023
Quality Review of Documents Working Group meeting with Interested parties	Labelling	Industry Stakeholders	29/11/2022 22/02/2023 30/05/2023 06/11/2023
Name Review Group	Name review	Industry Stakeholders	16/11/2023
Protection of personal data and commercially confidential information (CCI) for documents uploaded and published in the Clinical Trials Information System (CTIS): Workshop on draft guidance	Clinical trials	Industry stakeholders	14/07/22
Big Data Steering Group and industry	Big data	Industry stakeholders	30/05/22

Event name	Topic	Participants	Frequency
stakeholders meeting			
Biannual Big Data Steering Group and industry stakeholders meeting	Big data	Industry stakeholders	26/05/23
Clinical Data Publication (Policy 0070) re-launch - EMA webinar	Clinical trials data	Industry stakeholders	16/05/23
Reform of the EU pharmaceutical legislation for SMEs	Reform EU pharmaceutical legislation	Small medium sized enterprises	24/11/23

Overview of stakeholder events involving industry stakeholders held in 2022-2023

Meeting date	Meeting title	Stakeholder type	Frequency
2022 annual meeting of the members and Coordinating Group of the European network of paediatric research at the EMA (Enpr-EMA)	Innovation, paediatric medicines	Multi-stakeholders	04/10/22
Annual workshop of the European network of paediatric research at EMA (Enpr-EMA)	Innovation, paediatric medicines	Multi-stakeholders	10/10/23
9th paediatric strategy forum for medicinal product development in MAPK pathway inhibitors	Innovation, paediatric medicines	Multi-stakeholders	28-29/03/22
10th Paediatric strategy forum for DNA damage pathway inhibitors	Innovation, paediatric medicines	Multi-stakeholders	27-28/10/22

Meeting date	Meeting title	Stakeholder type	Frequency
12th Twelfth Paediatric Oncology Strategy Forum for Medicinal Product Development of CDK4/6, CDK7 and CDK9 Inhibitors in children and adolescents	Innovation, paediatric medicines	Multi-stakeholders	26-27/10/23
Multi stakeholder workshop on EMA's extended mandate	Shortages, public health emergencies	Multi-stakeholders	01/04/22
HMA/EMA multi-stakeholder workshop on shortages	Shortages, public health emergencies	Multi-stakeholders	01/03/23-02/03/23
ACT EU multi-stakeholder meeting on decentralised clinical trials	Clinical trials	Multi-stakeholders	04/10/22
ACT EU multi-stakeholder platform kick-off workshop	Clinical trials	Multi-stakeholders	22-06/23-23/06/23
ACT EU PA04 - Multi-stakeholder Workshop on ICH E6 R3 - Public Consultation	Clinical trials	Multi-stakeholders	13/07/23-14/07/23
ACT EU PA08 multi-stakeholder methodology workshop	Clinical trials	Multi-stakeholders	23/11/23
Multi-stakeholder workshop: Patient experience data in medicines development and regulatory decision-making	Patients experience data	Multi-stakeholders	21/06/22
Joint Heads of Medicines Agencies (HMA)/European Medicines Agency	Artificial Intelligence	Multi-stakeholders	20/11/23-21/11/23

Meeting date	Meeting title	Stakeholder type	Frequency
(EMA) AI workshop – Smart regulation in a rapidly evolving world			
EIC / EMA Info Day: Regulatory support for the development of innovative medicines and technologies	Innovation	Multi-stakeholders	31/01/23
Regulatory and scientific virtual conference on RNA-based medicines	Innovation	Multi-stakeholders	02/02/23
Shaping a European innovation ecosystem: EU-Innovation network multi-stakeholder meeting	Innovation	Multi-stakeholders	26/09/23
Strengthening life-sciences innovation across Europe: EU-Innovation Network conference	Innovation	Multi-stakeholders	21/11/23
Workshop on generating clinical evidence for treatment and prevention options for long-COVID / post-acute sequelae condition (PASC)	COVID-19/Long covid	Multi-stakeholders	17/11/23
DARWIN EU: multi-stakeholder information webinar	Big data	Multi-stakeholders	24/02/22
LinkedIn Live interview with Peter Arlett: Real-world evidence in medicines regulation	Big data	Multi-stakeholders	20/04/23
EMA multi-stakeholder	Big data/innovation	Multi-stakeholders	14-18/04/23

Meeting date	Meeting title	Stakeholder type	Frequency
workshop on qualification of novel methodologies			
Multi-stakeholder workshop on Real World Data (RWD) quality and Real World Evidence (RWE) use	Big data/innovation	Multi-stakeholders	26-27/06/23
SAFe Quarterly System Demos	SAFe products Quarterly system demo	Multi-stakeholders	Quarterly system demo - Q1 2022 Quarterly system demo - Q2 2022 Quarterly system demo - Q3 2022 Quarterly system demo - Q4 2022 Quarterly system demo - Q1 2023 Quarterly system demo - Q2 2023 Quarterly system demo - Q3 2023 Quarterly system demo - Q4 2023
Product lifecycle management (PLM)	Substance, product, organisation and referential (SPOR)	Multi-stakeholders	Webinar on requesting access to and using EMA's substance, product, organisation and referential (SPOR) application programming interface (API) (18/03/2022) Webinar on requesting access to and using EMA's substance, product, organisation and referential (SPOR) application

Meeting date	Meeting title	Stakeholder type	Frequency
			programming interface (API) (11/10/2022) SPOR and XEVMPD Week (17/04/2023) SPOR and xEVMPD Stakeholder Engagement Webinars : SPOR Data Governance (02/10/2023) SPOR and xEVMPD Stakeholder Engagement Webinars : Referentials Management Service (RMS) (03/10/2023) SPOR and xEVMPD Stakeholder Engagement Webinars : Organisation Management Service (OMS) (04/10/2023) SPOR and xEVMPD Stakeholder Engagement Webinars : Substance Management Service (SMS) (05/10/2023) SPOR and xEVMPD Stakeholder Engagement Webinars : Service Desk for SPOR and XEVMPD (10/10/2023) SPOR and xEVMPD Stakeholder

Meeting date	Meeting title	Stakeholder type	Frequency
			Engagement Webinars : Product Management Service (XEVMPD) (10/10/2023) SPOR and xEVMPD Stakeholder Engagement Webinars : EMA Account Management (11/10/2023) SPOR and xEVMPD Stakeholder Engagement Webinars : Substance, product, organisation and referential (SPOR) application programming interface (API) - SPOR API (12/10/2023)
Introduction to Organisation Management Service (OMS) and Referentials Management Service (RMS) services and activities: Industry webinar	SPOR	"Veterinary" Industry stakeholders	10/03/22
Product lifecycle management (PLM)	eAF Web Forms	Industry stakeholders	Introducing DADI: webinar on the digital application dataset integration (DADI) network project to replace electronic application forms (18/01/2022) Digital application dataset integration

Meeting date	Meeting title	Stakeholder type	Frequency
			(DADI) - common factors in the Fast Healthcare Interoperability Resources (FHIR) data standard for Article 57(2) and electronic application forms (eAF) (25/01/2022) DADI-PMS Webinar "Variations form for human medicinal products - What will happen at go-live" (16/05/2022) DADI Q&A Webinar - Variations form for Human Medicinal Products Go-live - (12/07/2022) DADI PDF electronic application forms (eAF) training webinar (26/07/2022) DADI PDF electronic application forms eAF training webinar (2/09/2022) Human Variations Form go-live Q&A session (27/10/2022) Human Variations web-based eAF Form training session (8/11/2022)

Meeting date	Meeting title	Stakeholder type	Frequency
			Human Variation eAF Q&A Clinic - session 1 (15/11/2022) Human Variation eAF Q&A Clinic - session 2 (22/11/2022) Human Variation eAF Q&A Clinic - session 3 (access management) (29/11/2022) Human Variations web-based eAF Form training session (15/12/2022) Human Variation eAF Q&A Clinic - session 4 (14/12/2022) Human Variation eAF Q&A Clinic - session 5 (19/12/2022) Human Variation eAF Q&A Clinic - session 6 (17/01/2023) Human Variation eAF Q&A Clinic - session 7 (22/01/2023) Human Variations web-based eAF Form training session (02/2/2023) Update on Human Variations web based eAF implementation on

Meeting date	Meeting title	Stakeholder type	Frequency
			PLM Portal (06/11/2023) Product Lifecycle Management Value Stream Deep-Dive Webinar (30/11/23)
Product lifecycle management (PLM)	Product Management Service (PMS)	Industry stakeholders	Product Management Services (PMS) Sub-Groups (SG) Webinar (09/09/2022) SMEs introductory meeting (14/12/2022)
Product lifecycle management (PLM)	Referentials Management Service (RMS)	Industry stakeholders	Introduction to Organisation Management Service (OMS) and Referentials Management Service (RMS) services and activities: Industry webinar (10/03/2022) Introduction to Referentials Management Service (RMS): Industry webinar (21/09/2022)
Product lifecycle management (PLM)	Organisation Management Service (OMS)	Industry stakeholders	Introduction to Organisation Management Service (OMS): Industry webinar (15/09/2022)
Product lifecycle management (PLM)	Substance management Service (SMS)	Industry stakeholders	Introduction to Substance Management Service (SMS):

Meeting date	Meeting title	Stakeholder type	Frequency
			Industry webinar (06/09/2022)
Product lifecycle management (PLM)	IRIS	Industry stakeholders	IRIS for Good Pharmacovigilance practice (GVP) inspections training session for industry users (07/09/2022)
Union Pharmacovigilance Database:	Pharmacovigilance	"Veterinary" Industry stakeholders	Follow up webinar on collection and recording of suspected adverse events for veterinary medicinal products (18/01/2022) Follow up webinar on signal detection, evaluation and yearly reporting (19/01/2022) Follow up webinar for marketing authorisation holders (25/01/2022) Webinar on variations not requiring assessment (VNRAs) for marketing authorisation holders (08/09/2022) Refresher webinar on signal management overview (27/10/2022) Union Product Database – Volume of sales webinar for

Meeting date	Meeting title	Stakeholder type	Frequency
			UPD industry users (24/04/2023) Union Product Database – Product grouping and 3rd country product names Webinar for UPD Industry users (18/09/2023)
Product Lifecycle Management (PLM)	Product Lifecycle Management (PLM)	Industry stakeholders	Product Lifecycle Management (PLM) Value Stream Deep-Dive Webinar

Glossary

3RWP	3Rs Working Party
ACT EU	Accelerating Clinical Trials in the EU
ACT EU MSP	Accelerating Clinical Trials in the EU Multi-Stakeholder Platform
AESGP	Association of the European Self-Medication Industry
AHEG	Ad Hoc Expert Groups
AI	Artificial Intelligence
ATMP	Advanced Therapy Medicinal Products
AVC	Association of Veterinary Consultants
BDSG	Big Data Steering Group
BEUC	European Consumer Organisation
BMPWP	Biosimilar Medicinal Products Working Party
BWP	Biologics Working Party
CAT	Committee for Advanced Therapies
CECP	Clinical Evaluation Consultation procedure
CHMP	Committee for Medicinal Products for Human Use
CMDh	Co-ordination Group for Mutual Recognition and Decentralised procedures - Human
CMDS	Critical Medical Device Shortages
CMDv	Co-ordination Group for Mutual Recognition and Decentralised procedures -Veterinary
CNSWP	Central Nervous System Working Party
CP	Centralised Procedure
CRO	Contract Research Organisation
CTCG	Clinical Trials Coordination Group
CTD	Common Technical Document
CTIS	Clinical Trials Information System
CTR	Clinical Trials Regulation
CTTI	Clinical Trials Transformation Initiative
CVMP	Committee for Medicinal Products for Veterinary Use
CVSWP	Cardiovascular Working Party
DHPC	Direct Healthcare Professional Communications
eAF	electronic Application Form

ECDC	European Centre for Disease Prevention and Control
EEA	European Economic Area
EFPIA	The European Federation of Pharmaceutical Industries and Associations
EHN	European Heart Network
EIC	European Innovation Council
ELPA	European Liver Patient Association
EMA	European Medicines Agency
EMNR	European Medicines Regulatory Network
ENPR	European Network of Paediatric Research
EPAR	European Public Assessment Report
ePI	Electronic Product Information
ERA	Environmental Risk Assessment
ESMP	European Shortages Monitoring Platform
ETF	Emergency Task Force
EU	European Union
EUCOPE	European Confederation of Pharmaceutical Entrepreneurs
EU-IN	EU Innovation Network
EUnetHTA	EMA/European Network for Health Technology Assessment
FDA	Food and Drug Administration
FWG	Formulation Working Group
GCP	Good Clinical Practices
GMP	Good Manufacturing Practices
GVP	Good Pharmacovigilance Practices
HAEMWP	Haematology Working Party
HCPWP	Healthcare Professionals Working Party
HCP POG	Healthcare Professionals Policy Officers' Group
HERA	Health Emergency Preparedness Authority
HMA	Heads of Medicines Agencies
HTA	Health Technology Assessment
IDWP	Infectious Disease Working Party
IMI	Innovative Medicines Initiative
ISG	Industry Standing Group
IT	Information Technology

ITF	Innovation Task Force
IWG	Inspectors Working Group
JICF	Joint Industrial Cooperation Forum
MAA	Marketing Authorisation Applications
MSP AG	Multi-stakeholder Platform Advisory Group
MDSSG	Joint Industrial Cooperation Forum
MSSG	Executive Steering Group on Shortages and Safety of Medicinal Products
MWP	Methodology Working Party
NCA	National Competent Authority
NcWP	Non-clinical Working Party
NIOG	Nitrosamines Implementation Oversight Group
NIRS	Near Infrared Spectroscopy
NSOEG	Nitrosamines Safety Operational Expert Group
NRG	Name Review Group
OEG	Operational Expert Group
OncWP	Oncology Working Party
PCWP	Patients' and Consumers' Working Party
PDCO	Paediatric Committee
PEC	Patient Engagement Collective
PED	Patient Experience Data
PECP	Performance Evaluation Consultation Procedure
PFAS	Per-, poly- Fluorinated Alkyl Substances
PhV	Pharmacovigilance
PhVWP	Pharmacovigilance Working Party - Veterinary
PIP	Paediatric Investigation Plan
PLM	Product Lifecycle Management
PRAC	Pharmacovigilance Risk Assessment Committee
PRIME	Priority Medicines
QIG	Quality Innovation Group
QoNM	Qualification of Novel Methodologies
QRD	Quality Review of Documents
QWP	Quality Working Party
R&D	Research & Development

RIWP	Rheumatology/Immunology Working Party
RMM	Risk Minimisation Measures
RWD	Real World Data
RWE	Real World Evidence
SAFe	Scaled Agile Framework
SAG	Scientific Advisory Group
SMEs	Micro, Small and Medium-sized Enterprises
SPOR	Substance, Product, Organisation and Referential
TIF	Thalassaemia International Federation
UPD	Union Product Database
UPhD	Union Pharmacovigilance Database
VWP	Vaccines Working Party

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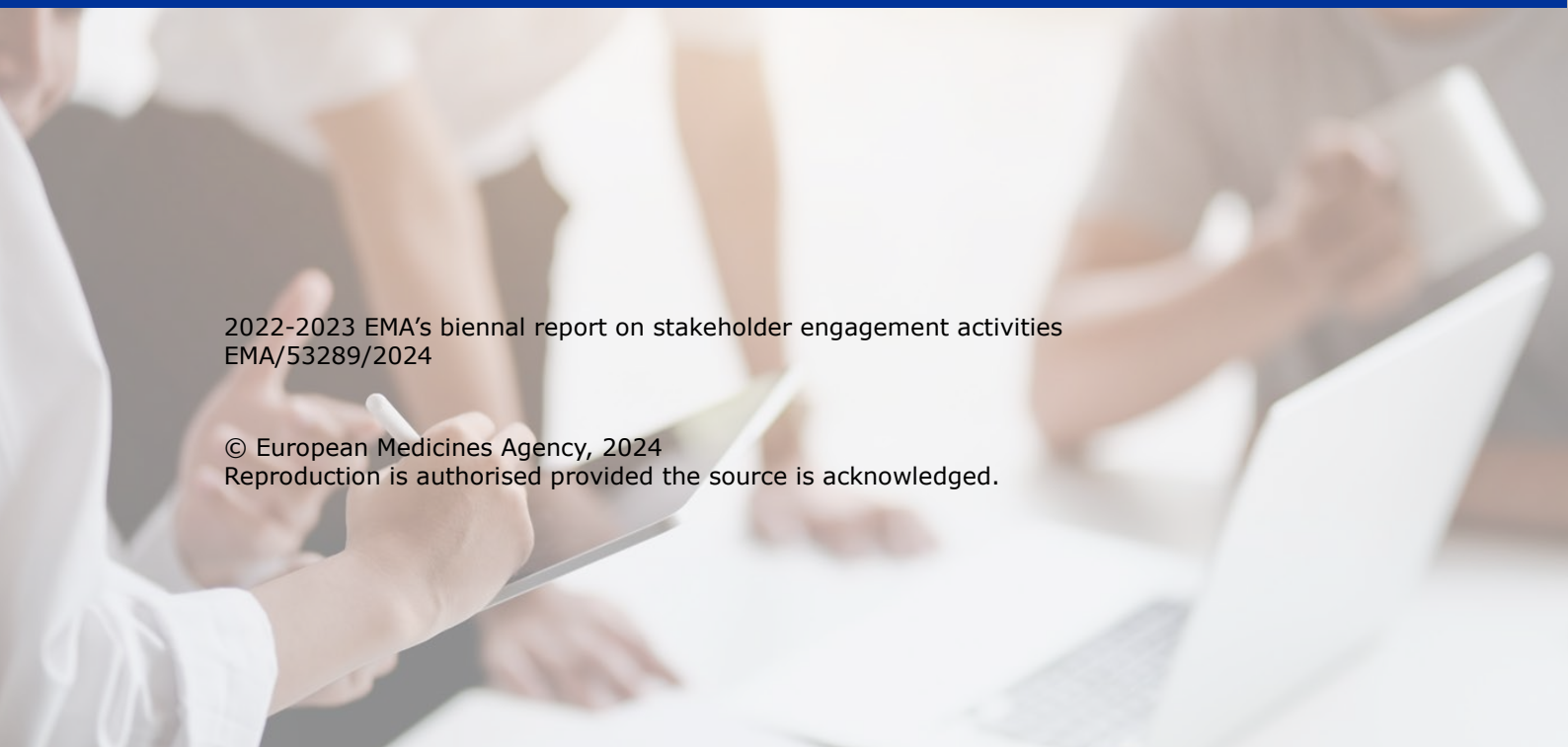

2022-2023 EMA's biennal report on stakeholder engagement activities
EMA/53289/2024