



22 July 2024
EMA/308177/2024
Stakeholders and Communication Division

Meeting summary – Patients and Consumers Working Party (PCWP) meeting

2 July 2024, hybrid meeting – WebEx/Room 2A

Co-Chairs: Juan Garcia-Burgos (EMA) and Marko Korenjak (PCWP)

1. Patient involvement at EMA

1.1. Update on patient involvement in Scientific Advice procedures

Kaisa Immonen (EMA) presented information concerning patient involvement in Scientific Advice procedures, which is one of the long-standing activities where patients have been involved at EMA. EMA's Stakeholder Engagement and Scientific Advice teams collaborated to investigate and discuss the reasons behind a recent decrease observed in the numbers of patients in these procedures. Several factors played a role, including a decrease in the number of submissions of scientific advice requests. Discussions are ongoing concerning a review of the template used by companies to submit requests for scientific advice to capture patient input more systematically. The PCWP will be kept updated on developments. For more information please see the [presentation](#) and the article on the [Added value of patients in Scientific Advice](#).

1.2. Activities involving patients/consumers during evaluation phase with CHMP

While patients are not currently members of the CHMP, several methodologies have been put in place to ensure that the patient voice is captured during the evaluation phase of the medicine regulatory pathway. Maria Mavris (EMA) described these different methodologies and provided updates on processes, which include Early dialogue with patient (and also healthcare professional) organisations at the start of the marketing authorisation procedure, participation in Scientific Advisory Groups and in oral explanations. Numbers of patients involved in the different activities were provided as well as information regarding updates of templates and processes. These activities have become more established and collaboration with the CHMP has increased to ensure stakeholder engagement all along the evaluation process. For more information please see the [presentation](#) and [process and FAQ document](#).

1.3. Remuneration of experts for involvement in EMA activities

Patients and healthcare professionals have voluntarily contributed their time and expertise to EMA in various activities. EMA will soon launch its call for expression of interest for these stakeholders to submit an expression of interest for the establishment of a pool of experts interested in involvement in EMA activities who may be remunerated for those activities. Maria Mavris (EMA) described the areas where



these stakeholders are involved, the different aspects of the remuneration including application, evaluation and selection and also emphasised the importance for the patient experts to provide more information about their activities and expertise to support their application, which will help in their selection to the pool. The call for expression of interest will be published on EMA's corporate website.

2. EMA International activities and patient engagement

2.1 International affairs: overview of activities

Michiel Hendrix (EMA) gave an overview of EMA in a global environment. International engagement is key to EMA's role and mandate, and international aspects cut across the organisation. Bilateral interactions make use of mechanisms for exchange of confidential information between regulators, for example in mutual recognition agreements, while multilateral interactions involve bodies such as ICH for international guidelines development and Instrument for Pre-accession Assistance (IPA), which builds capacity in EU candidate countries to bring their regulations into line with the EU. For more details and examples of projects, please see the [presentation](#).

2.2 Collaboration with US FDA

Maria Mavris (EMA) introduced the longstanding collaboration of EMA with the FDA, which was complemented by FDA liaison officer Katherine Tyner who presented her role. Katherine added that the FDA Patient Engagement Collaborative (PEC) was formed and modelled originally on the PCWP.

Maria then presented the patient engagement cluster, which was created in 2016 with FDA that now also includes Health Canada and aims to share best practices on involving patients along the medicines regulatory lifecycle and to further improve and extend the agencies' activities in this area. She then went on to present the PEC, which was formed in 2018 and with which the PCWP has held four joint annual meetings to date. PCWP members were invited to submit requests for the topic of the next annual meeting in 2025. For further details, please see the [presentation](#).

2.3 Progress on the establishment of the African Medicines Agency

Victoria Palmi Reig (EMA) presented on how EMA supports the establishment of the African Medicines Agency (AMA). EMA works in close coordination with the national competent authorities and activities focus on operationalisation and regulatory systems strengthening, including supporting the future governance and technical committees; strengthening scientific and regulatory expertise and bringing the experience of working together as a network; EMA is also coordinating and aligning European efforts in this international collaboration. Patient engagement at EMA was recently presented at a meeting with African regulators which was received with great interest, and there will be more opportunities to share learnings from these experiences and the added value of patient engagement as the AMA takes shape. For further details, please see the [presentation](#).

3. PCWP operations

3.1 Outcome of the survey on the joint PCWP/HCPWP workplan actions and discussion on PCWP-specific actions

A [work plan](#) is drafted and agreed with the working parties at the start of each mandate and covers both joint and individual working party activities. Ivana Silva (EMA) presented progress made at the mid-point of the 2022-2025 workplan during the joint meeting in [February 2024](#). To follow up on what direction to take regarding some identified actions, a survey was performed with both working parties and the findings were presented and discussed. In addition, areas including implementation of the new pharmaceutical legislation, mis/dis information, herbal medicines, polypharmacy, and non-clinical development emerged as topics for the next PCWP mandate. A progress update was also given on the

actions specific to the PCWP.

3.2 **Open discussion with working party members**

The working party held an open discussion on topics for consideration for future meetings and for inclusion in future mandates.

Meeting summary - Patients and Consumers Working Party (PCWP) and Healthcare Professionals Working Party (HCPWP) meeting

2 and 3 July 2024, hybrid meeting

Co-Chairs: Juan Garcia-Burgos (EMA), Marko Korenjak (PCWP) and Rosa Giuliani (HCPWP)

1. European Medicines Agency Network (EMAN) Strategy

1.1. Extension of the EMAN strategy to 2028

Melanie Carr (EMA) explained that as the current strategy runs to the end of 2025 and the review of the pharmaceutical legislation is pending finalisation, the decision has been made to update and extend the current strategy to 2028, when the new legislation is expected to become applicable. A mid-point report published in December 2023 showed the Network is broadly on track, and a joint workshop with HMAs was held earlier in 2024 to discuss focus areas for the updated strategy. While many topics are multiannual, new topics added include artificial intelligence, competitiveness and a regrouping of shortages with supply. A public consultation will be launched in late 2024 and the working parties will be kept updated. For more details, please see the [presentation](#).

2. Members' voice

2.1 PCO: Analysis on Medicines Overviews for CHMP opinions

François Houÿez (Eurordis) presented an analysis performed on Medicines Overviews for 179 CHMP opinions covering the period 2019-2023, both negative and positive, in rare diseases.

The objectives of the analysis were to i) analyse the type of evidence submitted to the CHMP (from randomised clinical trials and/or single arm trials), for rare diseases medicines (designated as orphan medicines or not) and ii) detect trends in causes over the last 5 years, compare failures and successes and explain the failures.

2.2 HCPO: Roles and Responsibilities of the GP in Prescribing in the Health Care System

Mary McCarthy (UEMO) presented on the many challenges associated with prescriptions by GPs, which are the biggest group of prescribers in health systems (see [presentation](#)).

Proposals for more multidisciplinary cooperation and agreement on prescribing patterns were then discussed, including shared care agreements and better regulations for safe prescribing. Shared care agreements would provide information to the GPs as to the specific risks to watch out for, the tests that would warrant referral back to the specialist, etc., and be shared with the patient. Generally, it was agreed that an increased collaboration between GPs and specialists was desirable, and that joint prescriptions of some drugs particularly for chronic pathologies should be considered. Indeed, the person who signs the prescription carries the risk and responsibility of that prescription and therefore specialist drugs, which lie outside the scope of general practitioners/family doctors, or unlicensed doses of medications, should only be prescribed in general practice/family medicine under a shared care agreement. Otherwise, the prescription risk and responsibility should be carried by specialists.

The importance of restricting the prescription of antibiotics more was also highlighted, with Scandinavia cited as an example to follow. It was agreed that point of care testing in GPs practices would be very

useful to reinforce prescribing decisions, providing that appropriate financing from health systems is secured.

The need for electronic systems communicating with each other in GP practices and hospitals, and accessible to patients, was also highlighted.

3. Clinical trial activities

3.1 Clinical trials raw data pilot - interim results

Marcia Rückbeil (EMA) presented an update on the raw data pilot, launched in July 2022 to assess whether using individual patient data from clinical trials (raw data) can help speed up and improve the medicine-evaluation process (see [presentation](#)). This update followed a [previous presentation](#) at the June 2022 PCWP/HCPWP meeting.

In this pilot, pharmaceutical companies submit raw data in an electronic structured format to EMA, on a voluntary basis, which allows regulators to more easily visualise and analyse the data if needed.

Expected benefits of the raw data analysis were presented, including the following benefits for patients and HCPs:

- Faster access to innovative, safe and effective medicines;
- Enhanced confidence in regulatory decision-making;
- Refined product labelling/ targeting of subgroups within the recommended indications;
- Facilitation of cross-product analysis.

The pilot is now halfway through with five procedures included in the pilot till December 2023 and surveys were conducted with pilot participants. Based on their feedback, an interim pilot report for the European Medicines Regulatory Network is being prepared, and a summary will be made available for the public in Q3 2024.

Preliminary learnings and recommendations for future implementation in the areas of regulatory assessment/ decision-making, capability and capacity, governance and processes, and technical aspects were presented. These include clear early signals confirming the added value of regulatory access to raw data in support of the regulatory assessment.

The pilot and its interim learnings were very positively received. In case of regular raw data submissions following the pilot, it was pointed out that communication tailored to patients and healthcare professionals would be beneficial to ensure confidence in the new process. Furthermore, it was clarified that data protection measures were put in place before data was received at EMA and analysed, and that the protection of personal data would continue to be prioritised in the next steps. Participants at the meeting highlighted the expected benefit of secondary use of data for additional cross-product analyses, referring to promising examples by other international regulators. The EMA project team encouraged meeting participants to share use cases on cross-product analyses.

3.2. Update on ACT EU and Multistakeholder Platform

Ana Zanoletty (EMA) presented an update on the Accelerating Clinical Trials in the European Union (ACT EU) initiative, launched in January 2022 by the European Commission, EMA and the Heads of Medicines Agencies (HMA) (see [presentation](#)). This initiative aims to improve the clinical trials environment in the EU/EEA through harmonisation, innovation and collaboration with stakeholders.

The update focused on two of the most recent ACT EU initiatives; support to non-commercial sponsors and the launch of two consolidated advice pilots. Regarding the consolidated advice pilots, a training and awareness session for regulators is planned on 15 July 2022, and a broadcast event for industry and non-commercial sponsors on 17 July 2022.

Denis Lacombe (EORTC), recently elected stakeholder co-chair of the multi-stakeholder platform advisory group (MSP AG), then presented the latest updates on the MSP, which is designed to facilitate dialogue between regulators and stakeholders regarding clinical trials. Benefits of the new Clinical Trials Information System (CTIS) and the Clinical Trials Regulation were also highlighted, as well as the various tools put in place for stakeholders to remain up to date with the ACT EU progress.

It was noted that interest from stakeholders for ACT EU is extremely high, all initiatives are very welcome, and future updates will be regularly provided to the PCWP/HCPWP.

4. Product Information

4.1. Update of the QRD template of the package leaflet

Monica Buch (EMA) provided an update from the QRD subgroup on the review of the package leaflet template. A second survey of stakeholders, including patients, consumers, and healthcare professionals regarding the potential inclusion of a key information section and benefits/risks balance information was conducted in February-March 2024.

Thorough discussions were held on 'key information section'. For the final decision the QRD Group considered among others, [EC's report on shortcomings in product information](#) based on the [PILS-BOX study](#) and [PIL-S study](#), EMA action plan related to the European Commission's recommendations on product information (point 6 on potential key information section in the SmPC and package leaflet) and the [draft report of the European parliament \(2023/0132\(COD\)\)](#). The QRD subgroup also looked at other jurisdictions such as MHRA, Australia, Academia and literature research, the industry position on having a "key information section" and the results of stakeholders surveys on the key information section. Based on all this information, they agreed to work on the inclusion of a "key information section" in the package leaflet.

Four models were discussed with industry and the QRD subgroup, however, the QRD subgroup ultimately decided to draft a model based on feedback from patients, consumers, and healthcare professionals. This will be a concise section at the beginning of the package leaflet with only key messages on the goal of treatment, main benefits, serious side effects, and main risks. The information should be factual and non-promotional and should not be a summary of the package leaflet. Cross-references to relevant sections and an instruction to read the rest of the PL should also be included. Please see the [presentation](#) for further details.

The draft proposal was presented and endorsed by the QRD group during their plenary meeting in June 2024. The final proposal of the draft template will go for public consultation, tentative date of October 2024. User testing of the new template with stakeholders will be considered an iterative process. Once the public consultation is closed, a multistakeholder workshop will be held to consider the feedback received. The timings of these activities will be confirmed.

5. Revision of EMA Guideline on Risk Assessment of Medicines on Reproduction and Lactation

5.1. Concept paper on the revision of the Guideline on Risk Assessment of Medicinal Products on Human Reproduction and Lactation: from Data to Labelling

Dinah Duarte (EMA) presented the concept paper, prepared by a CHMP/PRAC multi-stakeholder drafting group, and currently undergoing public consultation. (see [presentation](#))

The objective is to update the existing guideline to strengthen systematic generation of information on the benefits and risks of medicines in pregnancy and breastfeeding and consider developments in the non-clinical field and post-authorisation data, among others.

Karel Allegaert (EAP), member of the drafting group, underlined the importance of this update to the guideline, as proposed in the concept paper.

PCWP and HCPWP members were invited to contribute to the [public consultation](#) by 31 Aug 2024 and further disseminate this opportunity amongst their constituencies.

6. Transparency activities

6.1. Re-start of Clinical Data Publication

Silvia Garrido-Lestache (EMA) provided an update on the publication of clinical data for medicines. EMA has resumed its clinical data publication activities (policy 0070), which involves making clinical data submitted to the Agency as part of a regulatory procedure publicly available. This decision was made in line with the Management Board's decision in December 2022, after temporarily suspending these activities except for COVID-19 medicines.

As of September 2023, all initial marketing authorisation applications for new active substances will be subject to publication on the clinical data publication portal, regardless of whether they receive a positive or negative opinion, or whether the application is withdrawn.

EMA has implemented some changes to improve the efficiency of the clinical data publication process. These include sending invitation letters with detailed information about early request packages, use of the new anonymisation report form and offering the possibility of a pre-submission meeting. Additionally, updated Q&A guidance has been published to address practical questions related to procedural matters such as timelines, commercially confidential information, and the anonymisation process. (see [presentation](#))

PCWP and HCPWP members were invited to contribute to the CDP portal-[survey](#) to provide feedback on the user satisfaction and usability of the [Clinical Data Portal](#).

6.2. Revised CTIS transparency rules

Francesca Scotti (EMA) provided an update on the transparency rules in the Clinical Trials Information System (CTIS) (see [presentation](#)). The benefits of publishing clinical trial information were highlighted, followed by the two portals on which information on clinical trials in the European Economic Area is published: the [EU Clinical Trials Register](#) (all trials authorised until January 2023) and the [CTIS public portal](#), (trials authorised since January 2022). The CTIS public portal now offers higher level of transparency, through the publication of clinical trials documents (e.g. protocol) and of further useful information (e.g. principal investigator's contact details).

The transparency milestones were presented, leading to the revised CTIS portal launched on 18 June 2024. The main changes made to the transparency rules are the following:

- The publication is focused on key documents of interest:
- The deferral functionality was removed, so that documents are published earlier.

These changes enable stakeholder faster and more efficient access to clinical trial information, including patients and HCPs, reduce the burden to CTIS users and help promote the conduct of clinical research in the EU/EEA. A demonstration of the portal was then presented and it was highlighted that more functionalities would become progressively available.

7. Update on biosimilars

7.1. Outcome of biosimilars surveys to patients, healthcare professionals, industry and Heads of Medicines Agencies (HMA)

Diederik de Cock (collaborating expert) and Rosa Gonzalez-Quevedo (EMA) presented the preliminary results of a survey on information needs concerning biosimilars medicines. The aim is to support a HMA national toolkit and inform the development of possible future information materials by EMA. Whilst the analysis of the results is still ongoing and some differences can be seen in the responses of different stakeholder groups, the results from patients, consumers and healthcare professionals indicate that there are knowledge gaps and that awareness of the EMA materials and the statement on interchangeability is less than optimal. Once the report of the survey is completed it will be shared with the working parties. For more details, please see the [presentation](#).

8. AOB

No other business was raised.

9. Update on shortage related activities

9.1. Presentation of the activities of the Joint Action Coordination and Harmonisation of the Existing Systems against Shortages of Medicines – European Network (Chessmen)

Domenico Di Giorgio (AIFA) and Nuno Simões (Infarmed) presented an update of the CHESSMEN Joint Action. The main goal is to identify and share best practices at national level, support and harmonise joint efforts and ensure sustainability of the actions. Communication, including dealing with miscommunication, is an important aspect. The work is done in close collaboration with EMA including the Task Force on Availability of Authorised Medicines, MSSG and the SPOC network. The joint action (JA) is now in its second half and communications have been set up via the website and social media via LinkedIn. The stakeholder engagement plan mainly targets national stakeholders, with each member state expected to engage stakeholders through their existing networks and platforms. For more details, please see the [presentation](#).

In the discussion it was clarified that the definition of shortages in Regulation 2022/123 is applied whilst the JA is working on harmonising its implementation and bringing more granularity to defining different kinds of shortages, e.g. manufacturing problems vs hospital tendering issues, so that notifications can be aligned, and data collected will be comparable. As the JA does not involve all member states, discussions are brought to the SPOC working party so that decisions can be implemented throughout the EU.

9.2. Report on progress of the Subgroup on the implementation of the Good practice guidance for patient and healthcare professional organisations on the prevention of shortages

Inga Abed (EMA) presented an update on the work carried out to implement and review the guidance. A subgroup has been set up to reflect on how to increase awareness and provide input on new initiatives and identify particular needs of patients, consumers and healthcare professionals in this area. Awareness

of the guidance is low and there is a need to promote it. Language was identified as a possible barrier and the group proposed translating the existing factsheet and to further distribute at national level. In addition, usability could be improved by making the recommendations more targeted to therapeutic areas, so they become more tangible. A webinar was proposed for the end of 2024 to share information and good practices.

EMA is planning a campaign on shortages to launch in Q4 2024, including online content, a media seminar and a social media campaign co-created with volunteers from eligible organisations. The working parties expressed their support for a webinar and organisations will be contacted for feedback and a call for participation. For more details, please see the [presentation](#).

9.3. Update and feedback on Multistakeholder workshop on Glucagon-Like Peptide-1 (GLP-1) receptor agonists (GLP-1 RAs) shortages

Klaus Kruttwig (EMA) presented an overview of the current shortage situation of GLP-1 RAs, the key messages recommended by MSSG and provided a brief summary on the main outcomes of the discussion of the multistakeholder workshop on GLP-1 RA shortages. (see [presentation](#))

Members were updated on results of ongoing activities aimed at mapping causes for the increased demand, namely a study focusing on analysing prescription data. EMA elaborated that a DARWIN EU drug utilisation study on GLP-1 Ras has been initiated. This study aims to provide an overview of the characteristics of patients prescribed a GLP-1 RA medicinal product and how these have changed over the past ten years. This will help contextualise which determinants might be driving the demand for GLP-1 RA vis-à-vis the observed shortage of medicines, including exploring comparative trends of prescription of other medicinal products used in diabetes and for weight management as well as patterns of off-label use. Further information can be found here: [DARWIN EU@ - Drug Utilisation Study on GLP-1 Receptor Agonists | HMA-EMA Catalogues of real-world data sources and studies \(europa.eu\)](#)

9.4. Report on “Union List of Critical Medicines” development activities

Joao Ferreira (EMA) provided a status update on the Union list development activities, particularly regarding Phase 2, which is taking place in 2024. He reported on the outcome of the targeted stakeholder consultation that was held from March to May 2024. (see [presentation](#))

Members questioned how the annual updates to the list and the potential cumulative increase of included medicines would continue to serve the purpose of criticality and it was clarified that medicines in the list need to meet specific inclusion criteria and future annual refinements will mainly focus on novel substances.

Several members underlined the importance of supporting input from patient organisations by providing a more user-friendly format of the list. Participants also underscored the need to continue to explain how the list is developed, what are other lists used in the context of health treats as well as to communicate on other measures intended to facilitate availability of all medicines and not only those included in the Union List of Critical Medicines.

10. Vaccine related activities

10.1. Reinitiation of the vaccines outreach strategy

Melanie Carr and Marco Cavaleri (EMA) presented an update on EMA’s vaccines outreach strategy (see [presentation](#)). The presentation covered the need for such a strategy including key outcomes from the

2024 analysis, its goal, objectives, focus areas and opportunities for collaboration. A proposal for a new communication material for vaccines, vaccine fact boxes, was also presented.

Participants then discussed who to involve in this initiative including students/ younger users; the reasons for vaccine hesitancy including the issue of access to vaccines; the challenges that face immunocompromised patients, with participants offering to provide the corresponding data to EMA; and the most suitable communication formats. Available portals with epidemiology/vaccination data were also mentioned, and a European Vaccination Week/ Day proposed.

10.2. Presentation of the Vaccine Monitoring Platform

Alessandra Buoninfante and Catherine Cohet (EMA) presented on the Vaccine Monitoring Platform (VMP), which is a joint EMA-ECDC platform aiming to generate real-world evidence on the safety and effectiveness of vaccines in Europe, launched in 2022 (see [presentation](#)). The presentation first covered the link of the EMA's Emergency Task Force (ETF) to the VMP, including its responsibilities, recommendations, and previous workshops on the safety of COVID-19 vaccines. There is a legal mandate to monitor medicines after authorisation to support the work of ETF, which, for vaccines targeting an emergency, is achieved by the VMP. The VMP remit, structure and research areas were then presented. The presentation also covered the pathways for real-world evidence to support the VMP, current EMA vaccine studies in DARWIN EU, and the VMP communication plan aiming at increasing transparency. Participants discussed the route of administration of vaccines and how vaccines can help with antimicrobial resistance.

10.3. Update on the European Vaccination Information Portal (EVIP)

Nicolas White (ECDC) presented an update of EVIP, which is an EU-branded online portal aimed mainly at the general public that complements information provided by NCAs and links to resources in each country. EVIP is currently being updated and improved. A recent study with focus groups in ten countries looked at information uptake, including seeking, usage and sharing behaviours, among the public and healthcare professionals. The results are still under analysis but indicate some knowledge gaps. The update will also include a dedicated section for healthcare professionals to support professional-patient interactions, and publication is planned for 2025. Working party members were invited to give feedback on the new content and formats. For more details, please see the [presentation](#). In the discussion it was noted that feedback would be useful on general elements that users want to see included in the portal to meet their information needs, but also user testing of the materials themselves. There is already a small group involved in reviewing EVIP content, which may need to be expanded. EMA will discuss next steps with ECDC and update the working parties.