21 September 2017
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Stakeholders and Communication Division

Minutes - EMA Human Scientific Committees' Working Parties with Patients' and Consumers' Organisations (PCWP) and Healthcare Professionals' Organisations (HCPWP) joint meeting

20 September 2017, 08:30hrs to 16:45hrs meeting room: 2A

Role	Name
Co-chairs:	Juan Garcia (EMA) and Kaisa Immonen (PCWP)
Present:	**PCWP members**: Alzheimer Europe (AE), European AIDS treatment Group (EATG); European Cancer Patient Coalition (ECPC); European Consumers' Organisation (BEUC); European Federation of Allergy and Airways Diseases Patients' Associations (EFA); European Federation of Neurological Associations (EFNA); European Heart Network (EHN); European Multiple Sclerosis Platform (EMSP); European Organisation for Rare Diseases (EURORDIS); European Patients' Forum (EPF); European Public Health Alliance (EPHA); Health Action International - Europe (HAI); International Alliance of Patients' Organizations (IAPO); International Patient Organisation for Primary Immunodeficiencies (IPOPI); Myeloma Patients Europe (MPE); Patients Network for Medical Research and Health (EGAN). **HCPWP members**: European Association for Clinical Pharmacology and Therapeutics (EACPT); European Academy of Paediatrics (EAP); European Association for the Study of Diabetes (EASD); European Association of Hospital Pharmacists (EAHP); European Association of Urology (EAU); European Federation of Internal Medicines (EFIM); European Forum for Primary Care (EFPC); European Hematology Association (EHA); European League Against Rheumatism (EULAR); European Society for Medical Oncology (ESMO); European Society of Cardiology (ESC); European Society of Endocrinology (ESE); European Society of Radiology (ESR); Pharmaceutical Group of the European Union (PGEU); Standing Committee of European Doctors (CPME); The European Specialists Nurses Organisations (ESNO). **Representatives from the Agency's Scientific Committees**: Committee for Advanced Therapies (CAT); Committee for Medicinal Products for Human Use (CHMP); Committee on Herbal Medicinal Products (HMPC); Paediatric Committee

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Role	Name
	(PDCO); Pharmacovigilance Risk Assessment Committee (PRAC). **Observers**: Spanish Agency of Medicines and Medical Devices (AEMPS); European Multiple Endocrine Neoplasia Alliance (EMENA); European Network of Fibromyalgia Associations (ENFA); European Society of Oncology Pharmacy (ESOP); International Bureau for Epilepsy (IBE); Thalassaemia International Federation (TIF); European Union of General Practitioners (UEMO); London School of Economics (LSE). **IPA II observers:** Bosnia and Herzegovina; Kosovo under UNSC Resolution 1244/99; Montenegro; Serbia; The former Yugoslav Republic of Macedonia.

Introduction

J. Garcia Burgos (EMA) welcomed all participants and presented apologies on behalf of Gonzalo Calvo (HCPWP co-chair).

He reminded all participants of the goal to have paperless meetings. Nevertheless, printouts could be made available upon request in advance of the meeting.

No conflicts of interests were declared in relation to the agenda items.

The agenda was adopted with no additional points to be covered under A.O.B.

1. Involvement in EMA Activities

1.1. EMA preparedness on Brexit

N. Wathion (EMA) gave an update on the EMA preparedness activities for relocation, highlighting the following aspects:

a) New location – a decision is expected to be taken on 20 November 2017. The European Commission (EC) is finalising its assessment of the bids submitted by nineteen candidate countries. All offers to host EMA are publically available and can be found in the European Council website. EMA has been asked by the EC to provide technical comments to the EC on the proposed layout, the proposed facilities and the relocation plan. The EC will finalise its assessment by 30 September and then will present to the European Council. EMA is working on a relocation timetable on the basis of what the different bids refer to be available on the 1st of April of 2019 and it is still premature to give any indication on how EMA will move. The overall priority will be to maintain all scientific committees' work and ensure the procedures they oversee continue as smoothly as possible.

EMA also looked very carefully into the accessibility of all 19 candidate host cities including flight connections and public transports. In particular, EMA looked into how easy it is to travel from the airport to the proposed EMA premises. EMA has also specifically looked at accommodation in walking distance to the premises. The overall priority is to provide the most optimal working conditions for all delegates and experts contributing to the Agency's work.

b) Staff retention – EMA has performed surveys in order to help the Agency prepare for staff losses and to improve planning for succession and knowledge transfer.

As a concluding remark, Noel pointed out to the significant impact the decision on a new location will have on EMA capacity to keep its public health mission.

Members requested additional clarification on the location voting process, the impact on patients' and healthcare professionals' involvement in the evaluation procedures and on the relationship with the UK regulator, MHRA.

It was clarified that the voting process consists of a first voting round, where each Member State can give three points to the preferred offer, two points to their second preferred option and one point to the offer which the Member State ranks third. If an offer receives 3 voting points from at least 14 member States, that offer is considered to be the selected one. If this is not achieved, the three offers which receive the highest number of points will proceed to the second voting round. In case of more than three offers receiving the highest number of points, all offers that have received the same highest score will go on to the second voting round. In the second voting round, each Member State has one vote (consisting of one voting point) that it can give to one of the three (or more) offers which have been chosen for the second voting round. If an offer receives 14 votes or more, hence representing the majority, it is considered to be the selected offer. If no offer receives 14 votes or more, the two offers which receive the highest number of votes will proceed to the third round. In case of a tie between three (or more) offers, these will all go on to a third voting round. In the third voting round, each Member State has one vote (consisting of one voting point) that it can give to one of the offers which have been chosen for the third voting round. An offer that receives the highest number of votes will be considered the selected offer. In case of a tie, the decision will be taken by the European Council Presidency drawing lots between the tied offers. The offer drawn will be considered the selected one.

In relation to the impact on patients' and healthcare professionals' involvement in EMA activities, it was clarified that EMA's business continuity plan (BCP), with principles and methodology endorsed by Management Board in June this year, sets out three categories of activities. Category 3 includes the lowest priority activities and covers governance and support activities such as corporate governance, audits, participation in and organisation of meetings and conferences. These have already been reduced. Category 2 includes public health and strategic activities such as the contributions to fight against antimicrobial resistance, collaboration with health technology assessment bodies and initiatives in the area of availability of medicines. The highest prioritised activities are Category 1 activities which are either directly related to the assessment and safety monitoring of medicines or vital for maintaining the infrastructure of the European medicines regulatory network. Patient and healthcare professional involvement are included under category 1 activities.

In 2018, EMA activities will need to be further reduced taking as a basis the suspended/reduced activities for 2017.

Finally, in relation to MHRA (and MVP), there is a legacy with products, which assessment has been led by UK and EMA is currently working on how to redistribute the workload from the UK in a scenario where it would have to be treated as a 'third country'. Several member States are already investing heavily on additional resources.

J. Garcia Burgos (EMA) closed this agenda item mentioning that members will be kept informed with the latest information.

1.2. Update on public hearing

J. Garcia Burgos (EMA) updated on the preparations for the Agency's first public hearing, which has been called upon in the context of the safety referral on medicines containing valproate (see presentation). The hearing will not only inform PRAC but will be an opportunity for all stakeholders to listen to each other. The outcome of this review is expected to change the current RMP and the existing measures. It is therefore important to ensure all affected are listened to. An application form

and guidelines were prepared in lay language, published and disseminated amongst EMA stakeholders. EMA also set up a process to review all applications, with specific criteria for the selection of speakers. Due to time restrictions, it will not be possible to accommodate more than 16 speakers.

EMA received 84 applications. A total of 32 individuals have requested to speak. Some speakers where asked to group with each other. EMA declined 7 out of 32 requests to speak. Although these will not be able to speak their contribution will be circulated in writing to PRAC and will be published together with all the other individual contributions after the hearing. The meeting will be chaired by the PRAC chair, Dr June Raine.

The public hearing is a major step in openness and transparency. EMA has been involving patients and healthcare professionals for a long time and this will provide an additional tool to make EMA as open as possible and allow for the committee to gather input from a wide range of stakeholders. EMA will publish and summary report of the hearing and will carry out a lessons learnt exercise.

A. van der Zeijden (IAPO/PRAC) reminded all members that it is always possible to feedback to PRAC and not just when there is a public hearing.

Members were reminded that the hearing would be broadcast live on EMA's website from 12:45 to 18:00 UK time on 26 September and available to watch afterwards (see: PRAC: 25-28 September 2017)

For more information, including the summary of safety concerns and list of specific questions for the public hearing, see Valproate and related substances.

1.3. PCWP/HCPWP work-plans 2018/19

I. Silva (EMA) reported on progress made since June and outlined the main areas of focus emerging from initial reflection by members of the drafting group.

A discussion followed and the main points made are summarised below:

- Appreciation for the possibility to provide comments in advance and for seeing there are common areas of interest; the difficulty resides on providing input on specific timelines for the two-year period covered by the work plan
- Importance to identify indicators to demonstrate the impact of the work to be done; in particular it would be relevant to define at what point are we satisfied/ what is success
- Importance to include as part of the work plan the improved cooperation between EMA and HTA bodies; align working parties work plan with the joint EMA/EUNetHTA work plan
- Relevance to include EMA collaboration with the European Reference Networks (ERNs)
- There are so many areas that could be covered including adherence and AMR; where do we stop?
- Need to focus and align with EMA priorities and interests of the organisations
- Ensure links to ongoing activities (e.g. ADR reporting survey)

It was agreed to circulate the topics already identified and allow for two weeks for more comments.

It was also acknowledged that due to the extent of the work needed to compile an inclusive work plan, flexibility on the timelines would be required. Members agreed that flexibility on final approval of the work-plan would be also needed in light of expected information on EMA relocation by 20 November.

1.4. EMA training and resources – next steps

M. Mavris (EMA) presented an analysis of the last nine years of the EMA training day for patients. This training aims at introducing patients to the Agency and its work and is organised on an annual basis since 2007 (see presentation). In particular, Maria outlined the content description and evolution over the years as well as numbers and affiliation of participants. Many participants trained in the EMA annual training day have subsequently been involved as Members of Management Board, scientific committees and working parties. They have also been involved as experts in several EMA activities including scientific advice, scientific advisory groups, review of documents and written consultations.

One participant asked how information on trained participants who have become active in regulatory activities at their national level (not just involved at EMA level) could be collected. A possible survey could be explored.

The presentation also covered other training resources at EMA namely dedicated pages on EMA website for Resources and Training for patients and healthcare professionals. These pages include links to training documents, recent relevant workshops, videos of training days, short explanatory videos (EMABasics) and external training initiatives.

Maria concluded by raising some ideas for continued training and different suggestions on the way forward emerged from the discussion:

- Capitalise further on EMABasics and videos by making it easier to find for EMA-naive users and by adding sub-titles in EU official languages
- Expand training content to provide more explanation on the different ways medicines are brought to the market
- Make better use of webinars as a training tool (e.g. as a way to drive forward public health discussions and address topics like antimicrobial resistance)
- Continue to update the EMA website to provide more friendly interfaces
- Combine experience from national competent authorities and patient/consumer organisations to develop content
- Design training activities to train the organisations and not only the individuals
- Write articles for national societies and their journals/ magazines to increase awareness about information available for healthcare professionals
- Send out calls for expressions of interests on the EMA training day together with some explanation of what would follow for those trained – a summary could be developed together with working party members

Next training will take place on 21 November and will be open to patients, consumers and will be also extended to healthcare professionals and some young people. A call for expressions of interest will be launched in October.

2. Committee feedback

2.1. Feedback from meeting with civil society representatives

M. Mavris (EMA) reported on the meeting organised on 7 July with civil society representatives on EMA scientific committees. As an outcome of the discussion, there was general support to the idea of

reviewing the EMA document on the role of patients and healthcare professionals in scientific committees. In addition, three key areas were identified for improved support to civil society representatives: preparation of member prior to each plenary meeting, induction and ongoing training, and feedback mechanisms.

A meeting with each of the Committee' chairs will be organised to build on the feedback received and a follow up meeting will take place next year.

One member suggested that this group of civil society representatives could be useful to prepare a proposal to the EC on how to promote participation of civil society in committees. EMA mentioned this should be done together with EC and as part of a joint collaboration to prepare future calls.

Another member highlighted the importance of clarifying that civil society in the context of EMA means patients, consumers and healthcare professionals.

Another member pointed out to the need to continue to explore how more people of the working parties could be involved in the work of the scientific committees.

Members agreed with the proposal to revise the EMA document on the role of civil society and launch a consultation amongst PCWP and HCPWP members.

2.2. Committee for Human Medicinal Products (CHMP)

C. Prieto (CHMP) gave an update on CHMP opinions and involvement of patients and healthcare professionals between June and September (see presentation).

One member requested clarification on the role of patient organisations in the PRIME process. It was mentioned that PRIME is using existing processes such as Scientific Advice (SA) where patients are not always involved. It was clarified that involvement should occur when questions of a clinical nature are put forward by the applicant requesting the advice.

Another member pointed out that even though patients are not involved in the selection of candidates for PRIME it would still be important to have their involvement in discussing what constitutes an unmet medical need and that patients should be involved in all related activities for products given a PRIME status.

It was agreed to invite EMA colleagues working on PRIME to present again and discuss further during a future PCWP/HCPWP meeting.

2.3. Committee for Advanced Therapies (CAT)

B. Gansbacher (CAT) gave an update on CAT activities covering marketing authorisations until July 2017. He pointed out to important technologies that are dominating CAT discussions: CAR T-cell therapy in leukaemia and improved genome editing technologies which allow for simple approach to genetic modification of cells, such as the CRISPR/Cas9 system. CAT will organise an expert meeting on gene-editing technology in October.

2.4. Herbal Medicinal Products Committee (HMPC)

S. Bager (HMPC) reported that the Committee is currently working on the aspects of purity and effects of pollution on herbals. He also mentioned that the pool of patients interested to work with the committee had all participated as observers during one meeting this year.

Collected feedback from the observers will be used to discuss the way forward with a possible presentation during the HMPC meeting in November.

2.5. Pharmacovigilance and Risk Assessment Committee (PRAC)

R. Anderson (PRAC) gave an update on safety referrals and signals (see presentation).

One member pointed to the amount of important signals identified by PRAC and inquired about how these are communicated to the medical community. EMA clarified that some signals may result in updates of the product information but targeted safety communications are prepared only in very specific cases, for example following the outcome of a safety referral.

2.6. Paediatric Committee (PDCO)

D. Athanasiou (PDCO) introduced himself as one of the new patient representatives at the PDCO. Dimitrios highlighted three challenges for the committee: extensive off label use in children of medicines approved only for use in adults; volume of Paediatric Investigation Plans (PIPs) and their impact on approval of medicines; how to follow up with the obligations that companies have.

3. Members voice

3.1. Patient registries

C. Thalheim (EMSP) shared the content of his presentation to the 11th EMA Pharmacovigilance stakeholders' forum, focusing on EMA's patient registries initiative and the experience in the field of multiple sclerosis. EMSP have mapped 20 MS registries which cover more than 200.000 patients. The overall idea is to move from drug specific registries to disease specific registries by facilitating interactions between regulators and registry holders, capitalise on networks of registries that are already developed or push for their development. Christoph pointed out that as part of this work, two EMA workshops were organised in the past months: one on Cystic fibrosis registries (June) and one on Multiple sclerosis registries (July); findings of these workshops will be included in a report to become available in the upcoming weeks. Next steps foresee the launch of an inventory of registries hosted on the ENCePP platform (http://www.encepp.eu/).

Christoph highlighted the importance of independent quality certification by or on behalf of EMA that may provide confidence in registry data and that investment should be put on something that is long-lasting.

3.2. EAHP current priorities

J. Peppard (EAHP) presented on the current activities of the European Association of Hospital Pharmacists. These include the European Statements of Hospital Pharmacy and examples of good practice and a common training framework for hospital pharmacy. AMR and shortages are two other topics where EAHP is actively engaged.

3.3. Motivation on ESNO's choice creating guideline on biosimilar for nurses

B. Oomen (ESNO) reported on their initiative to prepare a guide for nurses on biosimilars as a follow up to the EMA guide for healthcare professionals and called for members to consider their involvement in its drafting and possible endorsement. Document to be circulated once available.

4. Access to medicines

4.1. Compassionate use

Role of EMA and member states

C. Bouygues (EMA) presented on the regulatory and legal framework on compassionate use (see presentation).

Compassionate use (CU) is a mechanism enabling health care professionals in a European Member State (MS) to provide access to investigational products to patients with serious or life-threatening conditions who have no satisfactory alternative treatment options outside clinical trial setting, i.e. investigational products that have not yet been authorised by regulatory authorities. CU is not a substitute for off-label use or for not conducting clinical trials. There is substantial heterogeneity in EU with regard to requirements for CU programmes.

Regulation (EC) No 726/2004, specifically Article 83, provides the legal basis for making a medicine available for compassionate use at the European level. Although there have been nearly 12 years of experience, only 5 opinions have been issued. These opinions are issued by CHMP and are publically available on the EMA website. It is up to Member states to provide a framework to implement the outcome. At present, the EC expert group STAMP is reflecting on possible ways to optimise the existing regulatory tools.

Perspectives from patients and HCPs

F. Houyez (EURORDIS) shared the viewpoints from a patient organisation perspective, underlining that compassionate use programmes (CUP) are a response for patients with the most urgent need for a new therapeutic option (it is really for a well-defined population). No major cases where opening a compassionate use programme before the clinical trial completed recruitment has created a problem. Some compassionate use programmes have been used to test in real-life the risk minimisation measures before submitting a marketing application. The EU legislation includes a 'may' provision for EMA involvement. EURORDIS has published a position paper on compassionate use, calling for a revision of EMA guidelines on CUP. In addition, the paper provides 28 recommendations, including for patients and consumer organisations and for regulators.

One member commented that the main problem for healthcare professionals was to ensure patients, through them, got access to these medicines and enquired how to enforce or create rules to enforce companies to make them available.

EMA clarified that there is no way to force companies to start a CUP; EMA can discuss and find an agreement with the company however, for CHMP to issue an opinion, it needs the data from the company.

FDA is using expanded access studies which can then be used as part of their applications. The EMA/FDA rare diseases cluster is something created recently to share opportunities and challenges between the two agencies.

Another member inquired about the use of data generated during the CUP. The CUP is a treatment and there are difficulties in collecting high quality data (different from what is collected in the context of CT).

One member commented on the fact that CUP are reimbursed in France, however this is not the case for all countries in the EU.

There was overall support to present EURORDIS position paper on compassionate use to HMA.

EMA will explore a possible presentation to CHMP.

4.2. EMA already works successfully with patients and HTA organisations: But where are the payers, and who are they?

A. Schuurman (MEDEV) reported on the first meeting organised between EMA and European Union healthcare payers (19 September 2017). The aim was to explore synergies and foster mutual understanding and cooperation to help improve timely and affordable access of patients to new medicinal products. The meeting aimed to be complementary to EMA's existing cooperation with health technology assessment (HTA) bodies and especially with EUnetHTA.

Experience over the last decades has shown that delays in pricing and reimbursement negotiations at national and regional level can sometimes occur, because drug developers are primarily focused on demonstrating the safety, efficacy and quality of a medicine for regulatory assessment but often do not generate sufficient evidence for cost-effectiveness assessments and pricing and reimbursement decisions. As a consequence, more research is required post-authorisation.

An important area for collaboration between regulators and healthcare payers is information sharing. Timely, adequate information could streamline decision-making and facilitate faster access to appropriate care.

EMA and EU payers will consider organising follow-up meetings to explore the above and other areas of collaboration, and discuss how to create a more effective partnership in order to improve the exchange of knowledge and information between regulators and payers.

A report on the meeting will be published in due course.

An open debate followed the presentation with members expressing a commitment to work together and emphasising that PCWP and HCPWP are a unique resource for collaboration with regulators, HTA bodies and payers.

EMA will maintain members informed of developments related with EMA activities in this domain.

5. Organisational matters

5.1. Revision of PCWP/HCPWP mandates and rules of procedure

I. Silva (EMA) updated on progress since June. Ivana reminded that the aspects requiring revision included the simplification of the name of the working parties, the revision of the membership structure to allow participation of as many eligible organisations as possible, the implementation of proxy votes and other operational aspects around the running of elections and the streamlining of annual work plans.

EMA is currently working on such revision in order to:

- Maintain two separate documents to address the mandate, objectives and composition of each working party
  - Include additional provisions for clarity on nomination process and identification of members/alternates
  - Clarify the role of observers (permanent; ad-hoc)

- Create a common document covering the rules of procedure for PCWP and HCPWP
  - Move away from template structure used for the Scientific Committees' working parties
  - Include proxy vote only for elections
  - Include section on decision-making process

Members welcomed progress made. Final proposal will be presented to PCWP/HCPWP once agreed by EMA management and legal services.

5.2. Re-assessment of eligibility status of organisations

N. Bere (EMA) provided an update on progress made since June. Nathalie reminded that the scope of this exercise was the annual re-assessment of eligibility of organisations; no change was foreseen for initial requests for eligibility. Instead of the current annual submission and evaluation of documentation confirming eligibility criteria, the proposal for a streamlined re-assessment process includes an annual self-declaration of eligibility whereby organisations submit an electronic form confirming compliance with all 7 eligibility criteria and funding sources. After submission of their annual self-declaration and validation by EMA, a confirmation of compliance with the eligibility criteria is issued. This process will be complemented by a yearly random assessment covering 20% of each group of organisations (patient/consumers and healthcare professionals). Relevant 'criteria' and 'process' documents will need to be revised accordingly and adopted by EMA Management Board.

Members agreed with the proposal to cover 20% of organisations for each group (patients/consumers and HCPs) as part of random checks.

A final proposal will be presented to PCWP/HCPWP once agreed by EMA management and legal services.

6. Information on medicines

6.1. Update on next steps (follow up on EC report)

Juan mentioned that an impact analysis was made and this now needs to be discussed with the EC. The working parties (WPs) will be updated on what is specifically proposed. In any case the WPs will be at the core of the implementation.

7. AOB

Members pointed to the aspects of tweeting and re-tweeting, suggesting that EMA policy should be revised to allow EMA to retweet; this was noted and WPs will be updated on further progress.