12 September 2017
EMA/355452/2017
Stakeholders and Communication Division

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

27 June 2017, 08:30hrs to 17:30hrs – 28 June 2017, 09:00hrs to 13:30hrs
meeting room: 3E

Role	Name
Co-chairs:	Juan Garcia (EMA), Kaisa Immonen (PCWP) and Gonzalo Calvo (HCPWP)
Present:	**PCWP members**: AGE Platform Europe (AGE); 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 Institute of Women's Health (EIWH); European Multiple Sclerosis Platform (EMSP); European Organisation for Rare Diseases (EURORDIS); European Patients' Forum (EPF); European Prostate Cancer Coalition (EUomo); European Public Health Alliance (EPHA); Health Action International - Europe (HAI); International Alliance of Patients' Organizations (IAPO); International Diabetes Federation European Region (IDF Europe); International Patient Organisation for Primary Immunodeficiencies (IPOPI); Myeloma Patients Europe (MPE); Patients Network for Medical Research and Health (EGAN) **HCPWP members**: European Academy of Allergy and Clinical Immunology (EAACI); 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); European Union Geriatric Medicine Society (EUGMS); Pharmaceutical Group of the European Union (PGEU); Standing Committee of European Doctors (CPME); The

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Role	Name
	European Specialists Nurses Organisations (ESNO); United European Gastroenterology (UEG) **Representatives from the Agency's Scientific Committees**: Committee for Advanced Therapies (CAT); Committee for Medicinal Products for Human Use (CHMP); Committee for Orphan Medicinal Products (COMP); Committee on Herbal Medicinal Products (HMPC); Paediatric Committee (PDCO); Pharmacovigilance Risk Assessment Committee (PRAC) **Observers**: Spanish Agency of Medicines and Medical Devices (AEMPS); European Network of Fibromyalgia Associations (ENFA); European Society of Oncology Pharmacy (ESOP); GAMIAN-Europe; International Bureau for Epilepsy (IBE); Pain Alliance Europe (PAE); European Union of General Practitioners (UEMO); **IPA II observers:** Bosnia and Herzegovina; Kosovo under UNSC Resolution 1244/99; Serbia; **QRD observers:** Austrian Agency for Health and Food Safety (AGES); Medicines Evaluation Board (MEB); Norwegian Medicines Agency; Medical Products Agency (MPA); Medicines and Healthcare Products Regulatory Agency (MHRA)
Adobe Connect:	**QRD observers:** Institute Marketing Authorisation of Medicinal Products & LCM (Austria); Bulgarian Drug Agency; Agency for medicinal products and medical devices of Croatia (HALMED); Finnish Medicines Agency (FIMEA); Health Products Regulatory Authority (HPRA); Federal Institute for Drugs and Medical Devices (Germany); Italian Medicines Agency (AIFA); State Agency of Medicines of the Republic of Latvia; Maltese Medicines Authority; Medicines Evaluation Board (MEB); Norwegian Medicines Agency; Spanish Agency of Medicines and Medical Devices (AEMPS) **Smpc AG observers:** Paul-Ehrlich-Institute

Introduction

Juan Garcia Burgos (EMA), provided health and safety information and welcomed all participants.

1. Involvement in EMA activities

1.1. EMA preparedness on Brexit

Noel Wathion (Deputy Executive Director) gave an update on Brexit activities. He explained that the Agency is working towards a scenario whereby the EMA offices will be relocated to the new country host MS by April 2019 and the preparedness activities have this deadline in mind. There may be other kinds of transitional arrangements which could be considered but these will depend on the outcome of the ongoing discussions between the UK and the EU. The heads of state and government have agreed the criteria and process for relocating both the EMA and European Bank Authority (EBA). Member States (MS) have to submit their formal bids to the European Commission by the end of July; these will be assessed by the Commission, in consultation with the agency in terms of the Agency's technical requirements.

There are significant challenges confronting the Agency; the location is still unknown and thus it is not clear what facilities will be available (e.g. office capacity, travel, hotels, homes, schools, etc.) and what will need to be put in place. MS have been reaching out to EMA in preparation of their bids, and several

delegations have visited the EMA offices to fully appreciate what the needs are. The overall aim is to ensure continuity of operations prior to, during and after the relocation.

From its side, EMA has started to put in place business continuity measures; Brexit preparedness will require much input from EMA staff however, depending on where the agency relocates, there could be a significant loss in staff numbers. Of course staff can be replaced but this will take time especially for the more specialised skills, plus training on regulatory aspects could take years. Although we will strive to carry out business as usual, it has been necessary to prioritise certain areas of work for 2017 and also for the upcoming work-plan for 2018/19; priorities have been categorised into 3 levels; high, medium and low. The 2017 impact is currently still limited for stakeholders as it is more about internal work. However, for instance the web portal development has been put on hold for time being.

Another aspect that has to be considered is that currently up to 20% of the EMA work is carried out by the UK delegates and experts and this now needs to be compensated by the other MS. Two working groups have been created; one for human medicines and one for veterinary medicines, and they will determine how to manage the re-distribution of the current and future workload. Legal timelines still need to be met, the quality of the work cannot be compromised and there must be no delays in access to medicines.

Following the presentation there were several questions from the working party members, for example one participant enquired whether the status of the committee member representatives from civil society had been discussed. Noel replied that this had not yet been discussed, but that we will need to have a look at this and that it affects ~~all~~ also other agencies and institutions. There was also a question about who makes the decision on where the Agency will move to, and what part the EMA plays in the decision? Noel explained that EMA will not be taking the decision; after the deadline at the end of July for MS to put in their bids, the EC will carry out the assessment, and will probably consult EMA on certain technical aspects. The assessment will then be publically available, and on this basis the Head of States will have a political discussion at their meeting in October, followed by voting in November.

One member highlighted that when the Swedish agency moved offices it lost 70% of staff and had to prolong its assessment times. She enquired whether there is a possibility to prolong EU assessment timelines and could experts from EEA area participate in the EMA work. Noel explained that Norway and Iceland already participate in the Committees work, but that we cannot compromise on timelines.

Another member asked if there is an option to postpone the Agency's departure, as negotiations are still early? Noel highlighted that the EC had been very clear on the April 2019 deadline; of course the final outcome could be different but we are currently working towards this defined date.

Noel finalised by informing everyone that there is a dedicated section on EMA website on Brexit which is kept up to date as well as an EC webpage (links). In addition we will share any key updates with you by email and at each PCWP/HCPWP meeting.

1.2. Proposal for revision of PCWP/HCPWP mandates and rules of procedure

Ivana Silva (EMA) presented a proposal that aims to simply and clarify the mandates of the working parties.

The documents, describing the mandate, objectives and rules of procedure for the Patients' and Consumers' Working Party (PCWP) and for the Healthcare Professionals' Working Party (HCPWP), were last updated on 30 May 2013 and, whilst the spirit of the mandates and objectives remain unchanged,

a number of elements have emerged over the past years that require a reflection in terms of revision/update of these documents, particularly in relation to the current composition, rules of participation and rules of procedure guiding the activity of the working parties.

The proposals include:

1) A simplification of the name of the working parties: the current names 'European Medicines Agency Human Scientific Committees' Working Party with Patients' and Consumers' Organisations (PCWP)' and 'European Medicines Agency Human Scientific Committees' Working Party with Healthcare Professionals' Organisations (HPCWP)' are too long and difficult to implement in headings and text. In practice we refer to the working parties as 'EMA Patients' and Consumers' Working Party' and 'EMA Healthcare Professionals' Working Party', however this needs to be formalised.

2) A revision of the membership structure to allow participation of more eligible organisations: the current composition foresees 20 seats for organisations; there are however a larger number of eligible organisations. Current practice is to invite eligible organisations who are not part of the working party to attend the plenary meetings as observers when there is available budget to reimburse participation, but this can only be confirmed close to the meeting dates. Live broadcast of workshops and information sessions has been implemented and participation in working parties' topic groups has been extended to eligible organisations, however organisations continue to request a more stable arrangement to ensure they can participate in a meaningful way to the scheduled plenary meetings.

3) Implementation of proxy votes and other operational aspects concerning elections: the current rules of procedure do not foresee the possibility for proxy votes in the event an organisation may not be able to attend a meeting where an election is planned. Procedural steps guiding the running of elections should be expanded to provide additional clarity. Although decision-making within the working parties is usually on a consensus basis, having a proxy vote system in place may also be useful in the exceptional cases when a vote is necessary.

4) Streamlining annual work plans into a single document and simplification of its adoption process: the current rules require that an annual work programme is prepared and circulated to the EMA Scientific Committees for adoption. No reference is made to the possibility of having a single work programme for both working parties.

In summary, to address the above-mentioned points the following is proposed:

- Implement the simplified name of the working parties in the mandates. They remain cross-Committee working parties and this will continue to be reflected in the composition.
- Separate the document's more static components from those that may require more flexibility in terms of possible future updates (e.g. have the mandate and objectives as a stand-alone document with two annexes: 1) composition and rules of participation and 2) rules of procedure, including meeting frequency)
- Discuss a fair solution to allow equal and transparent opportunities of participation of more eligible organisations in plenary meetings (e.g. pre-established rota throughout 3-year mandate)
- Revise the procedure for election of the chairperson and include details within the rules of procedure, including the conditions for proxy vote
- Include a clear reference in the rules of procedure that, upon agreement from the working party, a single or combined annual work programmes may be adopted, as needed.

Following the presentation several questions were raised, for example one member enquired how many eligible organisations are there and how many are reimbursed per meeting. Ivana explained that there are currently 37 eligible patient/consumer organisations and 28 healthcare professional organisations and at each meeting we reimburse 20 members from each group and in addition between 3-6 observers.

Another member asked if the meetings could be streamed via adobe so that the other eligible organisations could participate. Ivana replied that this is something we would like to try out; a first step would be taken already when discussing the agenda item on product information, with13 different members joining the session remotely. We will ask them for feedback on how it went and further explore the practicalities of implementing it more extensively.

A further clarification was sought regarding how the observers would join the meetings. It was explained that the proposal is that organisations could request in advance to participate as observers and register for a longer period, e.g. one year. This would be based on a first come first served basis.

Ivana finalised the session by explaining that all of the above proposals will be drafted and sent to members. She asked all members if they agreed in principle with the name change, the proxy vote system and the proposal for a single work plan, to which all members agreed.

1.3. Principles and practical considerations for streamlining process for re-assessment of eligibility status of organisations

Sylvie Benefice (EMA) presented a proposal for simplification of the process for the annual review of eligibility for patient/consumer and healthcare professional organisations.

Sylvie highlighted the current process whereby new organisations complete and submit an application form together with all relevant documentation, which is then evaluated according to the adopted eligibly criteria. If all the criteria are met then the organisation is accepted as an EMA eligible organisation and is included on the list on the EMA website. Thereafter there is an annual financial review of all eligible organisations and a two-yearly full re-evaluation. The annual reviews are quite time consuming as all of the 67 currently eligible organisations have to be re-evaluated.

The new proposal suggests that the process should remain the same for new organisations applying for eligibility (initial full evaluation) but that for the annual re-evaluation, eligible organisations would complete a self-declaration form and submit this to EMA. This form will include tick boxes whereby each criterion has to be confirmed – they would then receive a confirmation of eligibility. The proposal also suggests that there would be a certain number of random checks of eligibility each year (e.g. 2 for each group).

This will allow a faster, resource saving process for both EMA and the eligible organisations. If this is agreed by members then it will be given to EMA management for review and approval; if agreed, then a revised criteria document would need to be adopted by the EMA Management Board.

After the presentation there were several questions; for example if only two organisations are checked per year this could mean that some organisations would not be checked for several years? EMA explained that the system would be based on trust and that if we find during the random checks that organisations are not complying with the criteria then we would revise the process. This is similar to the current competing interest policy. It was highlighted that the criteria requires that organisations are completely transparent and publish on their websites information on their finances.

One participant suggested that there should be more random checks per year. EMA accepted this proposal but highlighted that the number would need to be agreed by all.

Another participant enquired what would happen if an organisation is found to not be in compliance with the eligibility criteria. EMA responded that it would first talk with the organisation in question, but that essentially they would be removed from the list on the website.

There was also a question as to whether the imminent relocation would have an impact. EMA replied that only that for those organisations who have their head office and statutes in the UK. We will be approaching these organisations to discuss how to proceed.

Following the discussion, all the members showed their support to go ahead with the proposal, but with an increased number of random checks. EMA will prepare a final proposal to be shared with the group prior to implementation.

1.4. Work-plans 2018/19

Ivana Silva (EMA) presented a draft of the proposed outline work-plan for 2018/19. The aim is to look forward in a prospective manner and identify areas of common interest; it should be ambitious but also feasible, taking into account the potential limitations due to the imminent relocation of the Agency.

It is proposed that the working parties should be involved in drafting the work-plan as follows:

The compilation of the work-plan will be agreed during the current meeting; the next step will be to establish a drafting group among members; this small group of people willing to work on the draft work-plan will be looking at and take into account recommendations and outcomes from recent workshops and topic groups, especially those actions not yet acted upon. The findings will be presented and discussed during the next joint meeting (20 September) and a first draft of the work-plan will follow for comments by all members. The final draft document will be circulated and put for adoption by 20 November.

After the presentation, one of the members asked for more clarification on the role of the drafting group. Ivana explained that this group can consist of any of the members who are interested and available, bearing in mind the summer timing and that the work will consist of discussing the draft work-plan content via email and teleconference.

Another member requested that the topic of AMR to be added to the work-plan, which was agreed.

It was also mentioned to look at the issues surrounding counterfeit medicines, which can be considered.

Any members wishing to join the working group should highlight their interest within one week of this meeting; this will be included in the post-meeting table of actions.

1.5. Topic Groups update

Nathalie Bere (EMA) gave an update on the topic groups (see presentation).

Most of the PCWP and HCPWP topic groups initiated in 2015 have now concluded and a report compiling the work of these groups has been published, as a supplement of the 2016 annual report on EMA's interaction with patients, consumers, healthcare professionals and their organisations.

Nathalie highlighted the continuation of the group on the involvement of young people. Although the main objective of the group has been achieved; 'Principles on the involvement of young people in EMA activities' has been adopted by EMA management, the group will continue to work together regarding the preparation of 'training/support' materials, gathering information from young people on their preferred methods of interaction with the Agency.

The former 'social media' topic group will be re-launched as 'Digital Media and Health' with the following tasks to be covered over the period June 2017-June 2019:

- Social media: Raise awareness of social media practices amongst PCWP/HCPWP and EMA, with a particular focus on promoting interactions and exchange of information.
- mHealth: identify questions that need reflection at PCWP and HCPWP level
- Real world evidence: promote a better understanding of how real world evidence is incorporated into the evaluation of medicines (collection, use, interpretation for regulatory purposes, curation) in a language that is meaningful to patients and healthcare professionals.
- Biosimilars; this topic group contributed to the review of the EMA/EC guide for healthcare professionals, which has been published on 5 May. The topic group will now concentrate on dissemination and use of the guide as an education resource; future work will include the development of materials targeted to patients.

Depending on the actions included in the PCWP/HCPWP 2018-2019 work plan a new topic group could be established in relation to personalised medicine. Areas for this topic group could include: involvement in efforts to improve communication on EMA activities related to personalised medicine (e.g. update of EMA relevant webpage), training on the implications of personalised medicine (Health literacy/ informatics literacy) and opportunities to develop dialogue with stakeholders.

There were also several proposals by PCWP/HCPWP members for new topic groups;

1. Safe use of medicines during pregnancy and lactation (proposed by Hildrun Sundseth); a topic group was not considered the best way forward in this instance; however the participation of patients (PCWP) is proposed within upcoming EMA activities in this area and it is proposed that Hildrun is the link within the PCWP. Some of the activities include an expert workshop on the evaluation of medicine safety in pregnancy that will focus on long-term or delayed pregnancy outcomes and a stakeholder meeting in November that will focus on the new Good Vigilance Practice module on pregnancy.
2. Medicines for older people (proposed by Barbro Westerholm, Hildrun Sundseth & Jean-Pierre Baeyens); rather than creating a topic group it has been proposed that Barbro and Jean-Pierre become members of the CHMP Geriatric Expert Group (GEG) and are the links within the PCWP/HCPWP. The GEG is called upon by the CHMP to address specific issues related to medicines for older people, including for example active engagement in the development of guidelines (e.g. characterisation of patients on the basis of frailty, quality guidance for medicines for older people). Also, PRAC will seek input from the GEG in referrals related to requests for post authorisation monitoring in older people as well as consult the GEG on a geriatric Guideline on Good Pharmacovigilance Practices (GVP) module, which is currently under development. Moreover, as part of the CHMP Assessment Report geriatric pilot to improve product information, the GEG provides comments to focus the attention of the assessors on the relevance of the data in the dossier to the population expected to use the product. In addition, there is a need to identify and address gaps in regulatory and scientific knowledge to advance regulatory science and the age group +85 has been particularly identified as one where more efforts are required. It has been suggested to develop a draft document outlining these concerns that could receive endorsement by PCWP/HCPWP.
3. Synergies between regulators and HTA (proposed by Francois Houyez); actions are being explored on the potential format this interaction could take with EUNeHTA; not a topic group at this stage.

4. EMA qualification procedures (proposed by Francois Houyez); a topic group is not considered a suitable way forward however we would propose raising awareness by preparing and publishing an EMABasic and as well as a dedicated information sheet to raise awareness.

5. Compassionate Use Programmes (proposed by Francois Houyez); rather than creating a topic group we would suggest raising awareness on this topic by organising a dedicated session during the PCWP/HCPWP meeting of September 2017.

6. Pharmacovigilance (proposed by Francois Houyez); it is suggested to discuss potential activities to take forward with the EMA Pharmacovigilance team, e.g. stakeholders forum, impact assessment. Additionally, the option of a session "where are we today in Pharmacovigilance?" could be discussed within the drafting process of the PCWP/HCPWP work plan 2018-2019.

2. Access to medicines

2.1. Conditional marketing authorisation: 10 year report

Zigmars Sebris (EMA) gave an update on conditional marketing authorisation (CMA) particularly as last year marked 10 years since its introduction in 2006 (see presentation). The CHMP guideline on CMA was also updated in 2016 and it was felt that it was important to publish a report after 10 years' experience. There continues to be high interest in this tool, especially as it is linked with activities in the context of facilitating early access, e.g. PRIME, Scientific Advice.

CMA is a tool whereby a marketing authorisation can be granted without the usual less comprehensive data than usually, in cases where data is not immediately available but there is a high public health need and the benefits of early access outweigh the risks due to limited data. Confirmatory studies are requested to be performed after authorisation and they are subject to strict monitoring and annual renewal.

The report highlights many interesting and detailed facts and statistics so it was recommended that members have a look at it.

After the presentation there were some clarifications requested, such as whether CMA affects marketing exclusivity? Zigmars explained that exclusivity starts at time of authorisation and there is no difference whether it is CMA.

Another participant enquired as to the benefits and safety issues that have been identified after ten years. Zigmars responded that the most positive aspect information is that none of the authorised medicines had to be revoked, and no major safety issues were identified, also that while on average an CMA authorisation is given four years earlier than comprehensive data is generated (which is necessary for a standard normal authorisation).

A participant suggested that there should be a registry of all the studies that have been requested. EMA responded that this information is included within each authorisation, and that EMA is currently looking at how to add publish more of this information within the EPARs concerning the results of these studies.

2.2. EUnetHTA(European network for Health Technology Assessment)

Michelle Mujoomdar (EUnetHTA directorate) provided an overview of EUnetHTA activities (see presentation). EUnetHTA was established to create an effective and sustainable network for HTA across Europe with the aim to develop reliable, timely, transparent and transferable information to

contribute to HTAs in European countries. EUnetHTA was originally established as the EUnetHTA Collaboration 2009, followed by the EUnetHTA Joint Action 2010-2012, EUnetHTA Joint Action 2 2012-2015 and now EUnetHTA Joint Action 3 2016-2020.

EUnetHTA JA3 is coordinated by the Dutch national health institute and currently has 81 partners consisting of national, regional and non-for-profit agencies that produce or contribute to HTA. There are seven ongoing work packages (see presentation for details) looking towards creating a sustainable model across the EU and within WP4 (Joint Production) and 5 (Evidence Generation) they are specifically looking how patients and healthcare professionals can be involved.

During the WP4 kick off meeting in September 2016 they discussed how to improve patient involvement in the process of EUnetHTA assessments; how to enhance the impact of the patients' perspective and also how to share experiences that can help define concrete proposals for future use. Around half of HTA bodes have some experience on patient involvement, but their methodologies vary considerably and for many it is a new step. There was however, general agreement that involving patients and healthcare professionals during the scoping phase is particularly helpful as it can: ensure inclusion of patient relevant outcomes, consider quality of life, ethical, and social issues, to better understand the clinical condition, care pathway, and current treatments available, and to understand what constitutes a clinically meaningful difference. It was also agreed that the choice of method will depend on the topic and activity.

Within WP5 the group can build on experiences and lessons learnt from the "Shaping European Early Dialogues" (SEED) project and at the same time also consider new and different ways of involving patients, e.g. look at approaches used by other HTA agencies. EunetHTA would also like to learn from EMAs experience and discuss how to coordinate approaches for patient involvement in advance of a discussion during the WP5 meeting later this year.

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During the WP4 kick off meeting in September 2016 there was a focused discussion on how to improve patient involvement in the process of EUnetHTA assessments; how to enhance the impact of the patients' perspective and also how to share experiences which can help define concrete proposals for future use. Around half of HTA bodes have some experience on patient involvement, but their methodologies vary considerably and for many it is a new step. There was however, general agreement that involving patients and healthcare professionals during the scoping phase is particularly helpful as it can 1) ensure patient relevant outcomes, quality of life aspects, ethical, and social issues are included, 2) better understand the clinical condition, care pathway, and current treatments available, and 3) understand what constitutes a clinically meaningful difference. It was also agreed that the choice of method will depend on the topic and activity.

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After the presentation there were several questions; one member asked if most HTA's only work with individual patients, and how can patient organisations be involved? Michelle responded that they do engage with patient organisations, but mainly on an ad-hoc basis. She highlighted that joining this meeting is a good opportunity to see how and who would like to be involved.

Another member enquired how, in practical terms could the PCWP/HCPWP members work with EUnetHTA. Michelle explained that they would be happy to engage with any organisations who are interested and that they are welcome to contact her directly.

Francois Houyez (Eurordis) recommended that any patients/consumers or healthcare professionals organisations who are interested to join the stakeholder pool of the HTA Network use the following link: https://ec.europa.eu/health/technology_assessment/consultations/call_htastakeholderpool_en He explained that there are currently 9 patients and consumer organisations members: EPF, BEUC, HAI, EPHA, EIWH, Eurordis, ECPC, EFA and IDF-Europe, and also 10 healthcare professional organisations. Applications are screened on the basis of the eligibility criteria listed in the call for expression of interest document, such as the European level, legal entity, transparency register, etc.

Juan wrapped up the session by explaining that the EMA/EUnetHTA work plan is currently being drafted and will include information on fostering engagement by sharing experience of existing platforms.

2.3. HMA/EMA Taskforce on availability of authorised medicines

Brendan Cuddy (EMA) gave an overview of shortages, highlighting the various causes such as economic (e.g. due to price competition or lack of reimbursement), manufacturing or quality-related (e.g. non-compliance with good manufacturing practice (GMP) or due to defective medicines).

For manufacturing or quality reasons for shortages, which fall within the remit of EMA, the Agency has a long history of activity in this area. Over time it has developed a comprehensive set of tools and documents, including a shortage catalogue, to support regulatory authorities in addressing shortage situations due to GMP non-compliance/quality defects and these are available on the EMA website.

Brendan highlighted statistical information on shortages including how many shortages have been prevented, or reduced by improving shortage management, e.g. rotate stock, facilitate batch release, as well as discussing with healthcare professional on managing reduced supplies, use of alternatives etc.

A joint EMA/HMA task force has been set up to work on unavailability / shortages of medicines that has been recognised as a priority topic in the EU Medicine Agencies Network Strategy to 2020. The task force, co-chaired by Kirstin Raudsepp (Head of Estonian National Agency and Noel Wathion (EMA),will aim to assess reasons why authorised medicines are not marketed in MS, establish a set of definitions and metrics to enhance shortage management and develop communication strategies during shortages within the network and with other actors in the healthcare system. The group will also work closely with international partners in this area and the EC.

During the first meeting of taskforce last week: 3 areas of focus were identified : 1) marketing of authorised products and what can be done to help to make them available through a regulated

framework, 2) how to prevent supply chain disruptions, and 3) how to improve communication within the network and to stakeholders.

Several questions followed from the members, for example one participant enquired whether there a way to have a more systematic information exchange, to ensure the organisations are advised as early as possible. Brendan agreed that the current catalogue is limited to shortages related to medicines assessed by EMA. It is hoped that the taskforce will help to improve information sharing and will look at ways to include information on a single portal for all EU medicines, potentially through links to agencies.

Brendan explained that they welcome further engaging with organisations in these activities; some organisations have already participated; for example ESMO and Eurordis have prepared reports. Anyone else who is interested should contact the PCWP/HCPWP secretariat.

2.4. PRIME update after workshop

PRIME is a scheme launched by the European Medicines Agency (EMA) to enhance support for the development of medicines that target an unmet medical need. This voluntary scheme is based on enhanced interaction and early dialogue with developers of promising medicines, to optimise development plans and speed up evaluation so these medicines can reach patients earlier.

Zahra Hanaizi (EMA) presented an update after one year of experience of PRIME, which was launched in March 2016 and on a stakeholder meeting held at the Agency in May 2017 (see presentation).

So far there have been 108 requests for eligibility to PRIME, out of which 27 have been given access to PRIME which represents a 22% success rate; half were from SMEs. Every month a report is published, including the negative outcomes (without names) for transparency purposes. A full list of medicines that have been granted eligibility to PRIME is also updated every month and publish on the EMA website.

For eligibility to be met there must be an unmet medical need, the nonclinical data should support pharmacological rationale and the exploratory clinical data should include relevant endpoints. Comparable historical control data can also be presented. Some of the reasons behind the refusals are included in the presentation, which were often related to issues with robustness of data, insufficient effect size and being received at too late a stage.

Positive feedback was received during the recent stakeholder meeting together with a few areas identified for improvement, for example more guidance on eligibility, clarifications on interactions with rapporteurs and regular updates from applicants.

After the presentation there were several suggestions from the participants, such as providing more information on the data supporting the proof of concept for each product. Zahra explained that such data is confidential and that EMA can only publish the indication.

Another member highlighted that it can be confusing with several similar ongoing projects / initiatives related to tackling unmet needs. Zahra advised that there is an EMA webpage on research and development which provides an overview of all the projects/initiatives.

An additional member asked how many PRIME products have so far reached authorisation. Zahra replied that this had not yet occurred.

3. Pharmacovigilance

3.1. Additional monitoring impact analysis

Justina Januskiene (EMA) explained that within the ongoing pharmacovigilance impact analysis there will be several studies conducted to measure the impact of additional monitoring. One such study is a survey directed to patients and healthcare professionals on reporting adverse reactions (see presentation). The survey has been circulated for comments and aims at helping to understand awareness on arrangements for reporting adverse drug reactions (side effects) and how they are reported by patients/consumers and healthcare professionals. The results will be analysed and a report containing aggregated information will be provided to the European Commission and will be published. The survey will be translated into all EU languages and circulated to all members for further dissemination.

One member asked if it was still possible to make comments on the survey structure. Justina responded that there was still time to share any comments/suggestions.

It was also suggested to give at least 4-6 weeks to respond to the survey.

After some further discussion it was agreed that any further comments could be forwarded after the meeting for consideration and it was reiterated that the organisations support in sharing the survey once live would be much appreciated.

4. Cross Agency activities

4.1. EMA framework of collaboration with academia – action plan

Monica Ensini (EMA) gave an update on the progress of the Agency's collaboration with academia. She explained that academics have always been involved in the work of the Agency but that it needed to be better structured and reinforced. This need had been highlighted in the EU medicines agencies network strategy to 2020, the EMA work plan 2016-17 and also within the EC horizon 2020 framework programme.

The agency started the process that led to the adoption of the framework with various discussions, meetings and consultations with academic stakeholders, including a survey and a workshop. After extensive consultation, agreement and consensus by the EC and the EMA MB was finally reached on 16 March 2017 with the framework adoption.

The purpose of the framework is to formalise and structure the collaboration between EMA and academics within the EU system. It will cover areas of common interest in relation to medicines for human and veterinary use, through existing regulatory tools. Implementation will be guided by an action plan, which identifies priorities to make sure that the framework will provide EMA with access to expertise whilst keeping up to date with the latest advances in science.

The major objectives are; to raise awareness of the mandate and work of the EU medicines regulatory networks, to promote and further develop the regulatory support to foster the translation of academic research, ensure that the best scientific expertise and academic research is available to inform regulatory decision-making; and collaborate on areas of research on regulatory science, such as novel approaches, endpoints and methodologies etc.

We have developed EMA dedicated webpages; includes featured information and news specifically targeted to academics and also areas of particular interest.

After the presentation there were several questions from members, for example how can the EMA reach out to academics when organising a workshop. The agency responded that it has a database that includes a network of EU organisations, which we hope to enlarge once the framework is more widely known.

Another member also asked about plans to raise awareness about EMA and this new framework, here Monica explained that there is much work to do together to build educational materials that would be useful for initial and continuing professional development but which will also attract academics to the website. This is included within the action plan, and is one of the activities of convergence between the EC and NCAs.

A member enquired why we are only looking at the EU since the cutting edge science may be elsewhere and academics move around. EMA responded that the framework indeed took this into consideration since science is worldwide and collaboration should not be limited to the EU.

4.2. EMA training for patients – facts and figures

This topic was postponed until the September meeting.

5. Committee feedback

5.1. Committee for Human Medicinal Products (CHMP)

Fatima Ventura (CHMP member) gave an overview of some recent activities at the CHMP (see slides).

Fatima highlighted that since the finalisation of the recent pilot project to involve patients within the CHMP oral explanations, they have continued to be invited to join the discussions at the CHMP. One recent example is the participation of two patient representatives in the oral explanation for Adlumiz; their input was very positively perceived by the CHMP members with their contribution to the benefit/risk discussion being extremely valuable. The Committee is now much more accustomed to patients being present during its deliberations; there are more and more examples where patients contribute in a meaningful way not only to scientific advice, SAGs and other committees, but also directly within the CHMP plenary discussions and this is now developing into an established routine.

5.2. Committee for Orphan Medicinal Products (COMP)

The representative was unable to attend the meeting.

5.3. Committee for Advance Therapies (CAT)

Kieran Breen (CAT member) gave an overview of the work of the CAT; whose activities are related to gene therapy products, somatic cell therapy products and tissue engineering products; ATMPs (see presentation). Kieran highlighted the number of medicine that have been evaluated and explained about a new product approved last month that is a cell based product to treat knee cartilage deficits.

5.4. Committee for Herbal Medicinal Products (HMPC)

Steinar Madsen (HMPC member) gave an overview of the herbal committee (see presentation). The Committee has recently elected a new chair and vice-chair. The main challenges the committee faces are that EU monographs, which are not compulsory, can only be prepared after the herbal preparation has been in use for at least 30 years overall and within the EU for a least 15 years. The current EU list

only has 12 such entries. He also explained how patient representatives have been participating as observers during the past few meetings and later in the year there will be further discussion on how they can be more involved in the work of the committee.

5.5. Pharmacovigilance Risk Assessment Committee (PRAC)

Albert van der Zeijden (PRAC member) gave an overview of the recent activities of interest from the PRAC. He highlighted the first public hearing that will be held at the Agency on 26 September in relation to an ongoing referral procedure for Valproate. The aim will be to hear from the public on their views on the associated risks of taking Valproate and how best to minimise and raise awareness of these risks.

5.6. Paediatric Committee (PDCO)

The representative was unable to attend the meeting.

6. Members voice

6.1. Patient Access Partnership (PACT)

Stanimir Hasardzhiev (EPF) presented a new initiative that has been developed within EPF (see presentation). The Patient Access Partnership (PACT) is a patient-led multi-stakeholder network bringing together patients, healthcare professionals, industry and EU policy makers and institutions, with the aim to develop innovative solutions to reduce inequities in access to healthcare in the EU. He gave the example of Bulgaria where the average time for an EMA authorisation to reach its market is between 1.5 and 3 years. For life saving medicines this has a huge impact and this is just one example of vast inequalities across Europe.

6.2. Addressing the Challenge and Constraints of Insulin Sources and Supply (ACCISS)

M. Lepeska presented a three year study (2015-2017) on global barriers on access to insulin supported by the Leona M. and Harry B. Helmsley Charitable Trust and Stichting ICF (see presentation). The aim of the study is to improve life-expectancy and quality of life for people with diabetes requiring insulin by addressing inequities and inefficiencies in the global insulin market.

The study looked at the evidence base of the global insulin market and potential models of supply, policy, and intervention to overcome barriers to insulin access including a toolbox developed in collaboration with multiple stakeholders, to influence policy change and reduce or eliminate barriers to insulin access.

The first year of the study was concerned with mapping insulin on the market, the second year looked at the perceptions of people using insulin and this year the focus will be on creating tools to advance access, decrease cost and increase awareness on regulatory issues and on knowledge on biosimilars.

7. Product Information

7.1. European Commission (EC) report on the shortcomings of product information

Juan Garcia-Burgos (EMA) opened the session and explained that the EC's report provides a unique opportunity to address shortcomings and areas of concern in relation to the current product information. The report had been circulated to participants beforehand. http://ec.europa.eu/health/sites/health/files/files/documents/2017_03_report_smpc-pl_en.pdf

The PCWP/HCPWP members were joined by other interested groups, in particular representatives from national competent authorities (QRD members), members of the EMA SmPC advisory group, representatives from the EC and EMA relevant staff. The Co-Rapporteur of the previous revision of the EU readability guideline was also present at the discussion.

Kristina Kurgonaite (EC) presented the report (see presentation). The EC report stems from a legal obligation within Article 59(4) of Directive 2001/83/EC1 whereby the Commission should present to the European Parliament and the Council an assessment report on current shortcomings in the summary of product characteristics and the package leaflet and how they could be improved in order to better meet the needs of patients and healthcare professionals. Two external surveys were carried out and the results were taken into account when preparing the report. In addition, input was given by the pharmaceutical committee (MS) and the relevant EMA colleagues.

Kristina stressed that the Summary of Product Characteristics (SmPC) and the Package Leaflet (PL) are an integral part of a medicines authorisation; in the EU all medicines must have accessible information on their safe and appropriate use. In order for companies to implement this requirement, and to simplify the process, relevant guidelines and templates have been developed.

The report was adopted on 22 March 2017 and a number of recommendations were identified on how to improve and better meet the needs of patients and healthcare professionals (within the boundaries of existing legislation):

1. Improve the PL; especially patient's comprehension and readability, as well as design and lay-out (less problems identified in the SmPC)
2. Amend related guidelines and the QRD template to enhance readability of PL; revision of existing guidelines, more flexibility among different medicines in template and introduce guidance on translations in the existing guidelines
3. Improving patient input in developing and testing the PLs; improve input from patients by making user testing more iterative and at a later stage, which could be coordinated by authorities during the assessment
4. Promote and exchange of best practice; promote evidence based, tested best examples of PL design and of process
5. Explore the use of electronic media in the SmPC and PL; complementary to paper PL that is required by the legislation; both as an integrated part of care process and as a tool to inform patients and health care professionals on changes in the SmPC and PL; should be based on EMA work with multi-stakeholder approach
6. Explore possibility of 'key information' section in the SmPC and PL; more experience and evidence needs to be gathered and appropriate testing can be used to determine usefulness; use of Quick Response (QR) codes should also be considered.

In summary, the EC, NCAs and EMA will work together toward implementing recommendations to improve the PL and will ensure that key stakeholders are involved in all proposed actions.

After the presentation Juan proposed to run through each of the recommendations to gather feedback from the members:

1) Improve the PL

Comments/suggestions/questions from members:

- The opportunity to improve the PL is very much welcomed. Both format and content need to be addressed separately.
- Overall there was agreement on the need to improve flexibility of the current template, which at the moment is felt to be very rigid.
- Agreement of the high public expectations on the recommendations and the necessity to meet the needs of patients and healthcare professional.
- It will be very important to work with experts in communication to ensure the latest outcome research and trends in benefit-risk communications are integrated in the review.
- Need to align any change introduced to the PL to the SmPC.
- The average patient only sees the side effects, without always understanding the proportions presented. This is often a long description, and when people see so many side effects they may become reluctant to use the medicine, so some participants thought the PL should only include the serious side effects.
- Some participants indicated that the PL is frightening and includes too much information on safety. There was overall agreement on the need to improve information on the benefit of taking the medicines and what beneficial effects patients can expect.
- EMA clarified that there is a legal requirement for companies to carry out user-testing (with patients) on a proposed PL. In additional, EMA sends all PLs to patients for their review during the medicine's assessment. This is however a superficial review and is not a formal user-testing.
- Some participants asked to add a link on leaflets to websites with more information.
- It was suggested to develop talking points on difficult information that prescribers could use to explain it to patients. Key information section should be reflected in the SmPC too.
- Participants call for an improvement of section 4.6 of the SmPC.

2) Amend guidelines and the QRD template

- The current readability guideline was last revised in 2009 and it is felt that an update is needed. Information design is lacking; there is no guide on how information should be presented and this is a barrier to having good information. Templates tend to stifle innovative thinking; they need more flexibility. Industry tends to use the template as a means for box ticking; as long as the information is in right order they feel they have satisfied the requirements. They need to look beyond that and produce documents that are informative and accessible. The current guidance doesn't address that. There is a need to make sure guidance covers not only content (that should also be updated) but also design.

- General Practitioners asked to be involved in editing PLs; for example they get the questions from the patients, which generally tend to be related to the side effects, which necessitate a nuanced discussion about the balance between benefit and risk, etc.
- A study carried out recently in Portugal was mentioned, which showed that the order and how the content is presented is important; there is a need to understand what information patients want to know first.
- In another recent survey in Spain, Portugal and Italy, patients said that they read the PL but they are not able to find the information they need.
- It was suggested to consider adding additional elements such as quick guides and audio-visual information.
- Other participants thought we should not add more information to the PL; if patients would like more specific information they should be advised to go to their healthcare professionals.

3) Improving patient input in developing and testing of PLs – the aim is to improve the way patients give input to PLs during its preparation. The recommendation highlights the need for enhanced user-testing to ensure that information is useful, complete and easy to understand. It should be coordinated by the regulatory authorities and should not disrupt or delay the assessment / authorisation process.

Testing should be carried out by patients with no knowledge or training on regulatory affairs; ideally those with experience of living with the disease under discussion. Organisations confirmed that they have good outreach with patients and their carers.

- Language is also a challenge across Europe, and there is a need to look at translations and ensure the same level of information is maintained.
- Some organisations have a permanent panel of patients willing to participate in surveys, testing etc; they could be a good source to ask for input when developing new user testing methodology.
- Some participants emphasised that the PL should read like a story–and that additional work has to be done for a meaningful PL.
- Others highlighted the importance of including how to source additional information; and this will be different in different countries
- Healthcare professionals have also a role in reviewing PLs and this should be taken into account when developing a process of user-testing.
- User testing specialists should be involved and consulted in the process.

4) Promotion and exchange of best practice

- There are already examples of good practice in the websites of national competent authorities and all PLs of centrally authorised medicines are publicly available through the EMA website. However accessibility and the need for selection are to be addressed. It is not easy to pick which examples are best, and certain criteria will need to be developed.
- There is a need to design a platform for communicating and exchanging, and the possibility of making it interactive was mentioned.
- Some participants raised concerns that sharing good practice may somehow hinder the imagination of companies to come up with new solutions or new ways to express certain information. Careful consideration should be paid to this.

- EMA website includes training on how to review and prepare SmPC; including presentations on each section of the SmPC. Although accessibility of this tool is very limited because of the lack of a friendly and accessible webpage hosting the tool, the experience gained should be considered for PLs.

5) Electronic SmPC and PL formats

- Although it is acknowledge that digital is the main vehicle for transmitting information today, it should not replace paper version; not everyone has access to electronic devices. Both forms should remain available. EMA agreed and reiterated that the report highlights the need to keep paper version.
- EMA and EC agreed on the need for a multi-stakeholder approach to discuss key principles on use of electronic formats; a workshop with all parties involved could be a good starting point.
- Sweden has good examples of using electronic formats for product information, and experiences should be shared before agreeing on any principles. Other examples of initiatives mentioned are WEBRADR (phone apps), etc., where patients can receive updates – two way communications.
- It is important to do a mapping exercise of all the ongoing initiatives on this field and ensuring that all relevant initiatives have a voice and representation to such workshop, so that any outcome is meaningful and provides a basis for further progress.
- E-formats engage better with young people who are usually a group difficult to target when it comes to provide medicines information.

6) "Key information" section in the SmPC and PL **-** the recommendation is to explore key information that is currently being looked at by EMA within EPAR summaries.

- EMA is currently evaluating the feasibility and usefulness of adding a 'key fact box' to EPAR summaries, as a tool to allow patients and healthcare professionals to rapidly identify key safety messages balanced with information on benefits of medicines.
- As a second step, the experience on the above could provide evidence-based principles to guide whether 'key fact boxes' could also be added to the PL.
- Recent research showed headline section helps people navigate to key information they want. http://etheses.whiterose.ac.uk/8415/
- However some participants mentioned that it is quite difficult to pinpoint what are key points for patients; it depends on individual needs.
- Some participants highlighted that sometimes EPAR summary can be more useful for patients than the PL because it is a document that was built from scratch with the collaboration of patients and looking at their needs. It was stressed that the proposed review is a new opportunity to create a new PL, involving the patients from the beginning, and ensuring it properly addresses the needs of patients

Juan **summarised** the discussion confirming that there is evidence showing that patients do read the PL but that they are not getting the information they want/need in the right format; the PL should be easy to read whilst containing all the key information. Improvements should focus on both format and content; they should allow sufficient flexibility and should introduce more information on the benefits of the medicine to counter balance the risks. The current guideline should be revised to reflect these changes and addresses these needs. It is important to involve all stakeholders and tap into adequate expertise in benefit-risk communication, in user-testing, etc., to ensure the latest outcome research

and trends in benefit-risk communications are integrated in the review. Adequate link needs to be established with healthcare professionals, including GPs. Any change that is introduced in the PL needs to be reflected in the SmPC.

The need to incorporate electronic formats needs to be addressed, as a first step in a multi-stakeholder workshop. Such workshop should follow a comprehensive mapping of all current ongoing initiatives in the field, to ensure they are all represented and have a voice.

Next steps: Juan thanked everyone for their input and explained that EMA and EC will reflect on how best to move forward. EMA will perform an analysis of the timelines, technicalities and resources that will be needed to implement actions that have been suggested. This analysis will need to take into account the particularly challenging situation that the Agency is currently facing both in terms of staffing and in relation to the UK's withdrawal from the EU. He stressed this is a unique opportunity to improve information on medicines in the EU and that public expectations are very high. Therefore it is very important to plan and execute adequately, in collaboration with patients, HCPs and other stakeholders, to achieve the best results. This topic will be discussed again during the PCWP/HCPWP meeting in September.

8. A.O.B

8.1. PCWP/HCPWP information session on antimicrobial resistance

I. Silva (EMA) presented the draft agenda for the session on antimicrobial resistance (AMR) to be held on 19 September (1st day of joint meeting). The session expands on the ECDC presentation last meeting from Dominique Monnet and will take place in advance of the European Antibiotics Awareness day in November.

The aims are to raise awareness of EMA and ECDC work in the fight against AMR; enhance understanding of how EMA and ECDC support the overall European and global-level fight against AMR; discuss how to best coordinate efforts for awareness and empowerment by HCPWP and PCWP members.

The EC and WHO will provide an EU and global perspectives and EMA contributions will cover its activities on both human and veterinary fields.

The final section of the programme has been designed to bring on board contributions from organisations and we are seeking input to identify presenters. The day will end with a panel bringing together the views from EC, WHO, EMA, ECDC and also EFSA and members from the working parties. The event will be broadcast live.

Members provided positive feedback on the proposed agenda, highlighting the importance of covering research and development activities for antibiotics, communication aspects, education and clinical practice reality.

8.2. Public hearing

The first public hearing will be held on 26 September 2017, in the context of the ongoing review of valproate. On 11 July the registration process will open and we will send out all relevant information. This includes the summary of safety concerns, the application form to register either as speaker or observer and a general guidance booklet. EMAwill select speakers and observers on the basis of the applications received. Any member of the public can apply. The hearing will be broadcast live. EMA will reimburse speakers only.

PRAC members shared how important it is and how they are looking forward to it in anticipation; it will be very interesting to hear from the public. We see it from the PRAC scientific perspective, and the public hearing, together with other methods for public engagement (e.g. scientific advisory groups and written consultations) will complement PRAC's understanding of issues emerging from the real world setting.

Journalists will also take part. Participants pointed out that this should be used as an opportunity to raise awareness on pharmacovigilance processes and also on engagement in general.

Lessons learnt will be compiled for future refinement of the process.

The chairpersons thanked the participants for their contribution and participation in the meeting.

Close of meeting

Next PCWP/HCPWP joint meeting: 19-20 September 2017