26 January 2015
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Stakeholders and Communication Division

Minutes of the EMA Human Scientific Committees' Working Party with Patients' and Consumers' Organisations (PCWP) meeting with all eligible organisations

26 November 2014 – 09:00hrs to 16:30hrs – meeting room 2A

Role	Name
Co-chairs:	Isabelle Moulon (EMA) and David Haerry (PCWP)
Present:	**PCWP members:** AGE Platform Europe (AGE); Europa Uomo-The European Prostate Cancer Coalition (EUomo); 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 Public Health Alliance (EPHA); Health Action International Europe (HAI Europe); International Alliance of Patients' Organizations (IAPO); International Patient Organisation for Primary Immunodeficiencies (IPOPI); Patients Network for Medical Research and Health (EGAN) **Representatives from patient and consumer organisations:** European Haemophilia Consortium (EHC); European Lung Foundation (ELF); European Liver Patients Association (ELPA); European Network of Fibromyalgia Associates (ENFA); Global Alliance for Mental Illness Advocacy Networks (GAMIAN-Europe); International Bureau of Epilepsy (IBE); Myeloma Patients Europe (MPE); Pain Alliance Europe (PAE); SMA Europe (SMA E) **Representatives from the Agency's Scientific Committees:** Committee for Advanced Therapies (CAT); Committee on Herbal Medicinal Products (HMPC); Committee for Medicinal Products for Human Use (CHMP); Committee for Orphan Medicinal Products (COMP); Pharmacovigilance Risk Assessment Committee (PRAC) Observers: EMA Management Board; Co-ordination Group for Mutual Recognition & Decentralised Procedures – Human (CMD(h)); Healthcare Professionals' Working Party (HCPWP)

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Introduction

Isabelle Moulon (co-chair) welcomed the participants to the meeting. They were asked to declare any potential conflicts of interest in terms of the topics on the agenda and then relevant fire evacuation procedures were highlighted.

Isabelle introduced Maria Mavris who recently joined the EMA as a seconded expert from Eurordis and who will predominantly be working with Nathalie Bere on patient engagement.

The draft agenda was adopted with no additions.

1. Involvement of patient/consumer organisations in EMA activities

1.1. Overview of activities 2014

Nathalie Bere (EMA) provided an overview of the different activities where patients, consumers and their organisations have been involved in EMA activities during 2014, including comparison to previous years (see presentation).

This overview highlighted that patients and consumers continue to be an integral part of the Agency's work and are involved across a wide range of its activities throughout the medicines' life-cycle. The overall cases of involvement have again increased across the board (likely to be over 600 by the end of 2014, compared to 550 in 2013). They are members of many four of the committees at the EMA as well as the Patients and Consumers working Party (PCWP); a key forum for exchange of information between the agency and the organisations. Patients and consumers are systematically involved in the review of package leaflets, EPAR summaries and safety communications and participate in scientific advisory group meetings, scientific advice procedures, in the various conferences and workshops, often as speakers and have participated in several medicine-specific committee consultations. Within these activities they either participate as representatives of their organisations (general topics) or as individual disease-specific patient experts (product-related topics). In the latter case, they are treated as all other scientific experts and as such are required to complete a declaration of interests form which includes a confidentiality agreement.

This information will be included within the annual report on interactions between EMA, patients, consumers, healthcare professionals and their organisations, which will be presented to the EMA management board in 2015 and published on the EMA website thereafter.

1.2. Revision of the framework of interaction between EMA and patients/consumers

Isabelle Moulon presented an overview of the proposed revision of the framework of interaction between the Agency and patient/consumer organisations. This revision has already been presented and discussed in previous PCWP meetings and is ready for adoption by the EMA Management Board in December 2014 (see presentation).

The revision not only provides information on the implementation of the previous objectives, including the role of patients in EMA committees, their involvement in benefit/risk discussions and the training necessary to support them, but also highlights future goals and provides a complete picture of the Agency's interaction with patients and consumers in one overarching document. The format of the framework provides a core high-level section able to accommodate new legislative and strategic initiatives (*e.g.* EMA Road Map to 2020), with two detailed annexes; 1) an action plan and 2) an

overview of patient involvement, which can be updated/revised depending on future developments / needs.

There were several comments and questions from the meeting participants; one participant asked that we ensure general organisations are included within the document, not only disease specific ones.

Another participant enquired as to how patient experts are contacted. The Agency explained that the eligible organisations are usually the first port of call however as it is not always possible to locate a suitable patient in this way, the network of individual patients needs to be increased to cover a wider range of therapeutic areas. The EMA will carry out a gap analysis, through an open call for expression of interest to be published on the EMA website, and will look to establish a pool of individual patients within the different disease areas. The eligible organisations would continue to be contacted for their input in identifying suitable patient experts for specific activities.

A further question was whether these individual experts would have to abide by the EMA code of conduct and declare any potential conflicts of interest, to which the EMA responded that they will be treated as all experts and as such are required to declare any interests and sign a confidentiality undertaking.

It was also asked whether citizens' organisations could join the EMA as an eligible organisation. It was explained that any organisation can apply, via the application form on the EMA website, however they should bear in mind that they would have to fulfil the criteria and in particular demonstrate that they are active in the field of pharmaceuticals.

It was also mentioned that the Agency should look for more qualitative information and feedback in terms of the value of patient input. This was fully acknowledged and is on the EMA's action plan.

Once the revision is adopted it will be published on the EMA website and the link circulated to all eligible organisations.

1.3. Implementation of new eligibility criteria requirements

Nathalie Bere provided a reminder on the new elements of the recently revised 'eligibility criteria' for organisations applying to the EMA.

In order to allow current eligible organisations to adjust and implement any necessary changes, a transitional period of 18 months has been applied; all organisations will be expected to fully comply with the revised process by the end of 2015.

The revisions relate to the transparency criteria, as follows:

- Organisations should publish their registered statutes, annual financial information (including all source(s) of funding, both public and private), and information on their activities.
- Organisations are required to complete a 'funding sources' table with full information on their sources of income (industry and non-industry related); giving individual amounts (both in absolute terms and in terms of overall percentage of the organisations total income) and submit this annually to the Agency, together with their official financial statement.
- In addition, organisations should follow a code of conduct/policy regulating their relationship with and independence from Pharmaceutical companies?

The Agency will evaluate the financial information as follows:

- Where industry-related funding exceeds 20% of the total income of the organisation, this must be from at least 3 separate companies and each individual contribution from a single company should not reach the majority of the organisation's total income.

There followed several questions from the group, such as the definition of 'industry?' The EMA clarified that it is defined as any individual company or association representing commercial manufacturers of medicines as well as healthcare products and services, including distributors and wholesalers, etc.

It was also asked which kinds of funding should be included, e.g. outside EU, from institutes, etc. The EMA confirmed that ALL funding from ANY sources should be included and that it is extremely important to include as much detail as possible.

The EMA proposed to prepare a guidance document with details on each of these criteria, how they should be met and how the information is evaluated.

Once the new requirements have been fully implemented the Agency will look at options to further simplify the procedure in the future.

1.4. Developments in EMA's communications – what's coming up?

Monika Benstetter gave an update on developments within the EMA's communications department (see presentation). Monika highlighted the different structures the Agency has in place to support its engagement and communication with stakeholders as well as its future plans, which include the development of a new EMA communications strategy with clearly identified objectives. A perception survey will be performed in 2015 with the overall aim of reviewing and adapting communication products and channels for the different stakeholder groups.

2015 will mark the 20th anniversary of the Agency, as well as 50 years of the EU pharmaceutical legislation and presents and excellent opportunity to re-focus communication, increase visibility and awareness and look to increase use of new channels and tools (social media, innovative communications, etc).

After the presentation it was asked whether patients would be included in the reviewing activities, to which the EMA confirmed that patients would be very much involved in the review and implementation processes.

1.5. EMA's collaboration with HTAs and Adaptive licensing

Jane Moseley gave an update on EMA's collaboration with Health Technology Assessment (HTA) bodies (see presentation).

HTA bodies provide recommendations on medicines that may be paid for or reimbursed by healthcare systems within each Member State. The Agency recognises that some new medicines are not reimbursed or used as expected after marketing authorisation therefore cooperation between regulators and HTA bodies has the potential to increase access to these medicines and reduce development costs.

The Agency has been working with the European Network for Health Technology Assessment (EUnetHTA), a network of EU organisations, relevant regional agencies and non-for-profit organisations that produce or contribute to HTA in Europe. The initial focus of the collaboration was a project looking into how the information on the benefits and risks of a medicine contained in European Public Assessment Reports (EPAR) could better address the needs of HTA bodies. The project has resulted in a series of improvements to the EPAR template. EMA-EUnetHTA also agreed a joint 3 year work plan (2013-2015).

In addition, the Agency includes HTA bodies within some of its scientific advice procedures. The aim of this is to allow medicine developers to gain feedback from regulators and HTA bodies at the same time, early in the development of a medicine (see link for further details).

Jane also provided an update on the EMA's ongoing pilot project on adaptive pathways. This is an approach to the authorisation of medicines that is currently being discussed and developed. Sometimes known as 'adaptive licensing', 'staggered approval' or 'progressive licensing', this work is part of its efforts to improve timely patient access to new medicines. It is particularly relevant for medicines with the potential to treat serious conditions where there is an unmet medical need (see link for further details).

In March 2014, the EMA published a call inviting companies to participate in a pilot project on adaptive pathways to help develop an understanding of how future adaptive pathways might be designed for different types of products and indications. As of November 2014, the Agency had received and assessed 29 applications as part of the pilot project, nine of which had been selected for discussion with the applicant. Stage I of the pilot project will close at the end of February 2015, followed by stage II which will include in-depth, face-to-face meetings with the applicants for the applications selected.

After 28 February 2015, EMA will still consider new applications for stage II face-to-face meetings if they are well-developed. As the project progresses, the European Commission will examine the legal and policy aspects related to adaptive licensing, in collaboration with the EU Member States and in consultation with relevant stakeholders as necessary.

The group will be kept updated on future developments in these areas.

2. Involvement of patient representatives in EMA committees

2.1. Involvement of young people in the PDCO - update

Benjamin Pelle and Sofia Nordenmalm (EMA) presented the initial feedback received following the circulation of a questionnaire amongst PDCO members, in relation to the potential involvement of young people in the work of the PDCO (see presentation) and to strengthen the participation of members representing patient's organisations in the activities of the PDCO.

This follows recommendations outlined within a concept paper (2012) that highlighted the need for a framework of interaction between EMA, PDCO, children and/or adolescents and patients' organisations as well as an understanding of the expectations and role of children and adolescents to support PDCO activities and the development of clear criteria on which situations would benefit from consultation with these stakeholders.

The results received so far (43% response rate - the deadline to be extended), showed that overall PDCO members felt there was benefit to involve children and/or adolescents in the activities of the PDCO that this could be within several different activities, such as workshops on specific conditions, product-specific PDCO discussions and patient-related topics, guidelines etc. They felt that the target paediatric population should be primarily adolescents and children with specific conditions, but also healthy children. The majority felt that they should be involved on a limited basis, e.g. twice yearly according to school holiday time and that there was a need to also involve, along with children, their parents, carers or legal representatives in the activities of the PDCO. Some of the potential challenges in involving children and adolescents in the PDCO activities highlighted included language barrier, training and adjusted terminology, access to patients, need to define appropriate questions, travel to EMA if participation in person / missing school and defining a minimum age for involvement. These are in addition to the patients' agreement and availability.

With regards to the questions on strengthening the participation of members representing patient's organisations in the activities of the PDCO, some of the areas where it was felt that these members could contribute included acceptability/suitability of proposed formulation(s), study design/feasibility, therapeutic need, acceptability/suitability of proposed study endpoints and targeted population.

The results of the questionnaire will be presented during the PDCO plenary in December and a small working group will be established to look at the scope and objectives of the project. The PDCO will also look at circulating a questionnaire to children to elicit their views on taking medicines and taking part in clinical trials. There are also plans to develop better information and communication on clinical trials to increase children's participation perhaps through a video or leaflet.

The group will be kept up to date on activities in this area and consulted on any proposed plans.

Some discussion followed the presentation; many agreeing that it is important to give young people a voice but that there are difficulties in doing this. It was mentioned that perhaps some experience within member states could be a source of information. The EMA agreed and will be liaising with authorities and other organisations that already have some experience in working with young people, e.g. UK, Poland.

2.2. Committees feedback

Some of the PCWP Committee representative members present at the meeting gave feedback on activities of interest to the patient groups.

The Herbal Committee (HMPC) representative, Steffen Bager, explained that their activities relate to the harmonisation of procedures and provisions concerning herbal medicinal products laid down in EU Member States, and further integrating herbal products in the European regulatory framework. As part of these objectives, the HMPC makes proposals for entries to a List of EU herbal substances, for adoption by the European Commission. It also creates herbal monographs which contain the view of the HMPC on the use of a medicine containing the herbal substance or preparation, e.g. what the herbal product is used for, who it is intended for and safety information such as undesirable effects and interactions with other medicines. They are published together with other documents, including an assessment report containing reviews of all available data relevant for the medicinal use of the herbal substance or preparation.

The HMPC has so far prepared over 130 monographs with over 150 herbal substances being assessed. The information is available on the EMA website.

The Committee for Advanced Therapies (CAT) representative, Keiran Breen, gave a short presentation of the CAT activities (see presentation). The CAT provides scientific recommendations on ATMP classifications, contributes to early discussions with developers via ATMP classifications, scientific-advice requests and collaborates with the Agency's Innovation Task Force. They also contribute to scientific advice for ATMPs and prepare scientific guidelines in the fields of gene- and cell-therapy and tissue-engineered products and evaluate quality and non-clinical data for certification procedures.

He gave an overview of the advanced therapy medicinal product (ATMP) applications received since 2009 as well as the recommendations on advanced therapy classifications and scientific advice, and on the revisions of the recently updated reflection paper on the classification of ATMPs.

The Committee for Orphan Medicinal Products (COMP) representative, Daniel O'Connor, gave an overview of the COMP activities (see presentation). The COMP is the committee that is responsible for reviewing orphan-medicinal-product designation. They are seeing an increase in the number of applications; 45 new applications in the November COMP and over 300 received throughout the year.

The COMP is currently finalising their work plan for 2015-18, drawing on discussions from the Rome presidency meeting.

The Committee for Human Medicinal Products (CHMP) representative, Harald Enzmann, gave an update on patient involvement in CHMP, specifically on the newly initiated pilot project to involve patients within some CHMP oral explanations. An increased involvement of patients within CHMP activities is a natural progression since patients are already systematically invited to participate within the scientific advisory group meetings (convened by the CHMP) - this project aims to provide an additional opportunity for the CHMP to obtain further inputs from the patient perspective when necessary. The overall objective is to facilitate a two-way discussion between EMA regulators and patients on outstanding issues related to the benefits and risks of a particular medicine under assessment. Harald emphasised that in order for this collaboration to be successful it is important that the patients who participate, do so in an independent manner, without any previous contacts with the pharmaceutical company involved and he called upon the patient organisations to help locate suitable patients.

The representative from the CMDh (Co-ordination Group for Mutual Recognition and Decentralised Procedures), Gabriele Eibenstein, explained that the Group is responsible for examining questions relating to a marketing authorisation of a medicine in two or more Member States, covering a variety of issues related to new applications, variations, renewals and pharmacovigilance activities. Following the implementation of the new pharmacovigilance legislation, the CMDh has been receiving many recommendations on nationally authorised products which is challenging due to the many differences often found within national authorisations, which need to be harmonised across Europe.

Finally, the Pharmacovigilance Risk Assessment Committee (PRAC) representative, Albert van der Zeijden, gave an overview of recent work within the committee and highlighted that patient involvement has increased significantly over recent years, especially with patients being members within the PRAC. He also explained that he and his alternate (Marco Greco) are increasingly being approached directly by patients wishing to share specific information with the PRAC for its consideration.

3. Pharmacovigilance

3.1. Adverse Drug Reaction (ADR) reporting

Steven Le Meur gave an update on Eudravigilance and Adverse Drug Reaction (ADR) reporting (see presentation). EudraVigilance is a system designed for collecting reports of suspected side effects. These reports are used for evaluating the benefits and risks of medicines during their development and monitoring their safety following authorisation.

The EudraVigilance Access Policy describes how stakeholders, such as national medicines regulatory authorities in EEA countries, healthcare professionals, patients and consumers, as well as marketing-authorisation holders and research organisations, can access information on suspected side effects submitted electronically to EudraVigilance. The www.adrreports.eu website provides aggregated data for suspected adverse drug reactions for Centrally Authorised Products (CAP) and has been available since May 2012.

The revision of the Eudravigilance access policy was recently under public consultation (until September 2014) and provided for an increased level of transparency and volume of information published online, the inclusion of line listings and access to a set of data fields from safety reports. Since 06 October 2014, information on suspected adverse drug reactions available on the database has been extended to include an additional 1,700 active substances contained in medicines approved at

national level in the European Union (EU). The update of the database also included the new leaflet for patients on how to report ADRs, in all languages, with links to national websites for direct reporting.

Steven presented some statistics showing that since the implementation of the new pharmcovigilance legislation and direct reporting of ADRs by patients there has been an increase in the number of reports received; overall and directly by patients.

3.2. Risk Management Plan (RMP) summary pilot feedback

Juan Garcia Burgos and Rosa Gonzalez-Quevedo (EMA) provided feedback on the ongoing RMP summary pilot project (see presentation).

A risk management plan is a document prepared for every medicine authorised in the EU. It describes what is known and not known about the safety of a medicine and the proposed measures to prevent or minimise the risks. It is a relatively long and complex document, written in technical language and is not made public.

The pilot proposal is to prepare RMP summaries that would summarise the information on risks and the measures to minimise such risks for each medicine to increase transparency and public access to relevant information on medicines. It is proposed to publish and target them to stakeholders and partners with a professional interest, but also to ensure that they remain clear information to the general public. The initial draft is provided by the company, further developed by the EMA during the assessment and then published on the EMA website at time of authorisation.

The one-year pilot phase began in March 2014 and to date 41 summaries have been published. The aim is to now gather feedback from patients and healthcare professionals on the usability of these documents. A short questionnaire will be circulated to representatives/members of patients' organisations and also to patients/members of the public not familiarised with the regulatory environment.

Post-meeting note: the questionnaire was sent out in December 2014.

3.3. Feedback from ISPE conference

Priya Bahri gave a brief overview of two presentations given during the International Conference on Pharmacoepidemiology & Therapeutic Risk Management in Taiwan in October (see presentation). The conference was organised by the International Society for Pharmacoepidemiology (ISPE) - a non-profit professional membership international organisation.

Priya presented during a plenary session on "How and why the European Medicines Agency uses patient input during the evaluation of medicines" and presented during a Symposium of Special Interest Group called BRACE (benefit-risk assessment, communication and evaluation) "How patients contribute to assessments at the EMA and messages for pharmacoepidemiology".

It was evident from the lively discussion that followed both presentation that the patients' voice is a recognised priority.

Projects

3.4. ADVANCE; project on vaccines

Xavier Kurz (EMA) presented the "Accelerated Development of Vaccine beNefit-risk Collaboration in Europe" (ADVANCE), which is an ongoing IMI (innovative medicines initiative) collaborative project

(see presentation). The aim is to monitor the benefits and risks of vaccines in Europe. Using the experience gained so far it hopes to build an efficient, sustainable, rapid monitoring system useful for all main stakeholders.

Xavier provided information on the first work package: "Development of best practice and code of conduct" which will develop and test a best practice guidance for the initiation, conduct and reporting of studies on the benefits and risks of vaccines in Europe. Four working groups have been created; i) Code of conduct, ii) Quality control and assurance, iii) Governance models and iv) Communication strategy and two workshops have been held to date; November 2013 and November 2014 (Representatives from PCWP 2014: Jose Drabwell and Marco Greco).

Post meeting note: slides prepared by Jose Drabwell were circulated after the meeting (she did not have the opportunity to present them during the meeting due to lack of time).

3.5. Collaboration at national level - survey

Maria Mavris presented a survey which has been prepared following PCWP organisations interest in further increasing interaction at member state level. The survey aims to gain an understanding of existing interactions between National Competent Authorities (NCA) and patient organisations via a web-based (Survey Monkey) multiple choice question / free text questionnaire.

Isabelle Moulon will present the questionnaire to the NCA professional communicators group for their input and help in circulating the questionnaire once ready.

The proposed questionnaire was circulated in the post-mail for comments.

4. A.O.B

The meeting dates for 2015 will be included within the post-mail and are included within the PCWP workplan which will be published early 2015.

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

Close of meeting

Next PCWP meeting: 4 March 2015 Joint PCWP/HCPWP meeting