4 August 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

3 June 2015 – 09:00hrs to 16:15hrs, meeting room 3E

Role	Name
Co-chairs:	Isabelle Moulon (EMA) and David Haerry (PCWP)
Present:	**PCWP members**: AGE Platform Europe (AGE); 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 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);Health Action International - Europe (HAI Europe); International Alliance of Patients' Organizations (IAPO); International Diabetes Federation European Region (IDF Europe); International Patient Organisation for Primary Immunodeficiencies (IPOPI); Patients Network for Medical Research and Health (EGAN) **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) **Speaker:** Health Research Authority (HRA), UK **Visiting expert:** Food and Drug Administration (FDA), USA **Observers**: EMA Management Board; Co-ordination Group for Mutual Recognition & Decentralised Procedures – Human (CMDh); Healthcare Professionals' Working Party (HCPWP); Global Alliance for Mental Illness Advocacy Networks (GAMIAN-Europe); Pain Alliance Europe (PAE)

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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 Andrea Furia-Helms who was a visiting expert from the FDA, here to observe how EMA engages with patients. She also introduced John Dowling a new representative from Europa Uomo.

The draft agenda was adopted.

1. Planning and reporting

1.1. Working methods for 2015 - Topic groups

Nathalie Bere (EMA) provided an overview of several PCWP topic groups which have recently been established within the following areas:

- Measure the impact of patient involvement in EMA activities
- Acknowledge and promote visibility of patient input in the Agency's activities
- Training
- Social Media
- Involvement of young people / children in EMA activities

Each of the EMA topic leaders then went through the aims and objectives of each of the topic groups for general agreement of all PCWP members.

Some comments from the PCWP members were as follows:

General: A proposal was made for an additional topic group focusing on older people – the EMA responded that this could perhaps be initiated during 2016, depending on how the other groups are progressing. There was also a suggestion for a group to look at the use of genetic data in medicine.

It was pointed out that there could be some overlap between the groups, e.g. groups 1 and 2; to which it was stressed that the groups are very flexible and that some joint work could be envisaged and that teleconferences could be held between several groups, as needed.

Measuring impact: It was highlighted that it should not just be about measuring outcomes but also on the process itself – to ensure that it is of value to all those involved.

Raising awareness and promote visibility: It was mentioned by several members in the room that this needs to be done better. There was a suggestion to have presentations available for use for patients going to conferences and events. It was also requested that EMA feeds back on organisations activities. Another member suggested that patient involvement at national level could be shared to promote practices.

Training: A recommendation was made that EUPATI should include EMA training.

Social Media: (joint PCWP/HCPWP): It was stated that there is a need to set limits for the group as this is such a huge area! There is also a need to look at how individuals are using social media – see how they can interact with EMA – direct reporting, quality etc.

Involvement of young people: It was emphasised that this relates to the involvement of young people but of course also parents, where applicable. One member highlighted an interesting project in the UK which looked at the different views between young people and their parents as well as the differences in information needs. This highlights the importance to look at national initiatives.

It was explained that the groups would be launched during the following weeks after the PCWP meeting, via TC, and where each group would need to nominate a co-topic leader from one of the organisations.

Each topic group will have its own timelines, meeting frequency and proposed outputs; however each group will be given the opportunity to feed back at the PCWP meetings.

2. Anniversary

2.1. Potential activities to mark the PCWP's 10th anniversary (2016)

Maria Mavris highlighted that next year will be the 10th anniversary of the creation of the PCWP and that the Agency would like to mark this important occasion with some celebrations!

Some suggestions include a dedicated 'celebratory' PCWP meeting where former co-chairs and members would be invited to participate. Another proposal is to publish an anniversary book (online) with text, photo and video contributions from members on the impact, importance and evolution of the working party.

In order to prepare for this event the Agency would like to set up a task force within the PCWP to help coordinate activities; a call for expression of interest will be circulated after the meeting.

Feedback from the group was that this is a very good idea and that it would also be a good way of promoting and raising awareness of patient involvement at the EMA. Social media could be used to circulate material and also a media campaign could be coordinated.

3. Involvement in EMA activities

3.1. Feedback from Scientific Committees

Daniel O'Connor, from the Committee for Orphan Medicinal Products (COMP), provided an update (see presentation). He explained that the COMP agendas and minutes are published on the EMA webpage and they provide an overview of the Committees ongoing work, including applications for orphan designation, requests for protocol assistance (scientific advice specifically for orphan medicines) and review of orphan designation at the time of marketing authorisation. He also gave an update on the COMP's significant benefit working group and an ongoing National Competent Authority (NCA)/COMP consultation on proposed process improvements for Orphan procedures. He mentioned that there is a proposal for a COMP workshop on significant benefit to be held in December.

Following the presentation, one of the participants asked if a medicine could lose its Orphan Drug status at the time of marketing authorisation if, for example, there has been a change in designation criteria. Daniel confirmed that at the time of marketing authorisation, the COMP re-assesses all the criteria for maintenance of orphan designation and this includes prevalence. He explained that significant benefit, defined in the Regulation as a "clinically relevant advantage or a major contribution to patient care", is one of the criteria for orphan designation that also has to be confirmed at the time of marketing authorisation. If the orphan status is not maintained (criteria for orphan designation are

no longer fulfilled), the medicine would still have a marketing authorisation but would not be eligible for the 10 year market exclusivity.

Anne Ambrose from the Coordination Group for Mutual Recognition and Decentralised Procedures (CMDh) gave a detailed overview of their work (see presentation). The CMDh was set up to examine questions relating to medicines authorised in two or more EU Member States through Mutual Recognition or Decentralised Procedures. If there is disagreement between Member States during the assessment of a medicine on grounds of potential serious risk to public health, the CMDh considers the matter and strives to reach an agreement. If this is not possible, the case is referred to the Committee for Medicinal Products for Human Use (CHMP) for arbitration.

The CMDh also looks at questions concerning the safety of non-centrally authorised medicines marketed in the EU. This includes adopting CMDh positions on safety-related EU referral procedures, taking account of the recommendations of the Pharmacovigilance Risk Assessment Committee (PRAC).

Each year the CMDh identifies a list of medicines for which harmonised product information should be drawn up, to promote the harmonisation of marketing authorisations across the EU.

Anne also highlighted some recent safety referrals and PRAC requests for advice which she felt would be of interest to the PCWP members.

Kieran Breen, from the Committee for Advanced Therapies (CAT), explained that since its creation the Committee has assessed a significant number of products for potential classification as advanced therapies. To date there are 5 advanced therapy medicines that have reached marketing approval.

Last year guidelines on what is an advanced therapy medicinal product were released for consultation and following extensive consultation, these have now been published and are available on the EMA website.

Nathalie Bere, in absence of Steffen Bager (the HMPC representative at the PCWP) explained that the HMPC were keen to explore if there were members of the PCWP who were potentially interested to be involved in some of the committee's activities, on a case-by-case basis as needed. It was suggested that a group of those interested in this topic could be formed and would be consulted as and when needed.

The PCWP members asked if it would be possible for Steffen to give a comprehensive overview of the HMPC work and where patient input could be considered. The participants felt that they would then be in a better position to respond to a request for interest.

3.2. PCWP involvement in preparation of Guidelines on the summary of clinical trial results for laypersons

Amanda Hunn from the UK Health Research Authority (HRA) joined the meeting to give a presentation on the development of guidelines on clinical trial results for laypersons, as referred within the recent clinical trial regulation; (EU) No 536/2014. The UK HRA will be leading in the preparation of this guidance and, following the request of the European Commission, the input of the PCWP will be sought.

Amanda gave an overview of the health authority and their previous experience in developing guidance and working with a range of stakeholders, including public dialogue, patient workshops including a study assessing what information participants felt should be available at the end of a study.

Work on this guidance will also build on existing work done by INVOLVE, NIHR and Harvard MRCT**,** such as lay summaries of protocol at the beginning of a study, initial review and assessment of plain English summaries and development of guidance on how to write a plain English summary.

The next steps will be to prepare a detailed work plan for delivery of the draft guidance by November 2015, to set up taskforce and work closely with interested stakeholders, including patients and consumers (PCWP sub-group), industry representatives and Academia.

After the presentation there followed several questions, comments and suggestions from the members, such as whether other modes of presenting results could also be investigated, e.g. voice, webcast, multi-media, etc, to which it was replied that this could indeed be explored as links to other materials could potentially be included within the summaries. One member also asked whether unfinished trials were also included, and it was confirmed that all trials have to be included. It was then also suggested to involve healthcare professionals in the review and to include guidance for sponsors.

The EMA advised that they would circulate a call for expression of interest to join a group to provide input to the development of the guidance after the meeting.

3.3. EUPATI; upcoming interaction guidances (regulatory, HTA, ethics, industry)

David Haerry gave an overview of a EUPATI initiative to produce some guidances for interaction with patients; he explained that guidance on meaningful and ethical interaction is missing in many areas and that as the EMA has developed a mature framework over time this could be used as a guiding principle (see presentation).

EUPATI aims to come up with guidance covering four areas: Industry, Ethics committees, Health Technology Assessment (HTA) and Regulators. These were discussed in more details at an interactive session within the DIA Europe meeting on April 15.

The first drafts are due early July 2015, with a report on the guidances due to IMI by January 2016. There will be external consultations during 2016 (EFPIA, EMA PCWP, EC) and they will also be sent to all EMA eligible organisations for comments.

3.4. Patient involvement in B/R at EMA; latest developments

Maria Mavris and Douwe Postmus gave an overview of a recent research project between the EMA and Groningen University in the Netherlands (see presentation). This project follows the EMA's ongoing initiatives to explore additional and complementary options for gathering patient input; in this case on their preferences to benefits and risks of treatments/medicines.

Patients are already involved in benefit/risk discussions at the EMA as members of the Pharmacovigilance committee (PRAC), through their participation in scientific advice/protocol assistance procedures and in scientific advisory group (SAG) meetings. There is also a pilot initiative involving patients in CHMP during oral explanations preceding decision-making

One of the objectives of the revised framework adopted in December 2014 by the EMA Management Board is to further enhance participation of patients and consumers in benefit/risk evaluation and this project is one methodology currently being tested to explore the feasibility and usefulness of eliciting patients values to inform the benefit-risk decision-making.

This feasibility project included melanoma and multiple myeloma patients and carers, as well as regulators from EMA. Its overall aim was to gather their preferences on treatment outcomes for a specific medicine; how much they valued a stated benefit compared to a potential risk, how they would prioritise these benefits and risks and also how much they would be prepared to gain and lose. There were two components to the study; an online questionnaire where individual preferences were collected, followed by a face to face discussion to gather group preferences.

The results will be shared with the conference organisers and participants (all data collected was anonymous). The outcomes were shared with the Committee for Human Medicinal Products (CHMP) to consider the use of this methodology during CHMP assessment (along with the others) to which they recommended the EMA trial further pilots.

4. Members voice; sharing practices

4.1. Working with young cancer patients

Rafal Swierzewski from the European cancer patient coalition gave a presentation on his work with young cancer patients in Poland (see presentation). He highlighted what children with cancer need and how organisations can help them. He explained how children are much more aware and knowledgeable on their condition and its symptoms and treatment than people realise and their knowledge and unique experience is underestimated. Childhood cancers are much more curable today but the number of cases increases as do the problems that patients and survivors face (e.g. follow-up treatment, behaviour, education, insurance, job opportunities etc.). Within youth groups the children are able to talk to each other and provide psychological support. Rafal gave an overview of some of the activities that the youth groups carry out, such as support to children and their families at paediatric oncology clinics, events at hospital wards, summer camps and educational programs to students, teachers and health professionals.

He finalised with some examples of potential involvement of young people at the EMA.

4.2. EUGenMed project

Hildrun Sundseth, from the European Institute of Women's Health presented a 2 year EU project within the European Union's Seventh Framework Programme for research called EUGenMed. The other partners of the project are Charité-University Medicine Berlin, Institute for Gender in Medicine (GIM) and Maastricht University. The project aims to develop a Roadmap for implementing sex and gender aspects into biomedical and health research and will build on existing activities.

Hildrun explained that both sex and gender matter in health; women live longer than men and the incidence and prevalence of diseases differ between men and women. Women have higher rates of osteoporosis, auto-immune diseases, eating disorder, Alzheimers, etc, while men have higher rates of Parkinsons, chronic liver disease, violence-related injuries, lung cancer, etc. Some diseases affect men and women differently, e.g. women can metabolise medicines differently. Therefore medicines would be safer and more effective for all if clinical research includes diverse population groups of all ages.

Translating the evidence from sex and gender research into regulatory practice will lead to more targeted, effective opportunities for prevention, diagnosis, treatment and care. The project has generated several recommendations (see presentation).

5. International experience

5.1. FDA engagement with patients

The EMA and the FDA recently carried out an exchange of information to share best practices on Patient Engagement in medicines development; an EMA staff member from the Patients and Consumers Department spent two weeks at the FDA offices in December 2014 to learn how they collaborate with patients and this was reciprocated with an FDA colleague joining the EMA for two weeks in June 2015.

The FDA colleague, Andrea Furia-Helms attended the PCWP meeting and presented the group with an overview of how the FDA works with patients (see presentation). She initiated her presentation with a high level explanation of the FDA's key roles, especially as there are some fundamental differences between the FDA and EMA procedures, such as the fact that the final decision to approve a product is taken 'in-house' by the FDA Division Directors, based on the review team's assessment and that the FDA also assesses foods and cosmetics as well as diagnostic and tobacco products.

The FDA patient liaison program coordinates the outreach and educational activities with patients, patient advocates and patient advocacy groups and makes the connection between the patients and the Review Teams. The Patient Representative Program incorporates patient/community advocates' voices into advisory committee and division discussions and Andrea explained how the patient representatives are selected and how they add value to FDA's decision making.

The importance of patient engagement for both Agencies was emphasised and that the success of the recent fellowships has led to a proposal to establish a platform for regular exchanges to share best practices and further learn from each other.

6. AOB

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

Close of meeting

Next PCWP meetings:

- Workshop on risk-minimisation measures: 16 September 2015
- PCWP/HCPWP joint meeting: 17 September 2015