EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

19 June 2015
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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

4 March 2015 – 09:00hrs to 17:15hrs, meeting room 3E

Role	Name
Co-chairs:	Isabelle Moulon (EMA), David Haerry (PCWP) and Gonzalo Calvo (HCPWP)
Present:	**PCWP members**: AGE Platform Europe (AGE); Alzheimer Europe (AE); European AIDS treatment Group (EATG); European Cancer Patient Coalition (ECPC); European Federation of Allergy and Airways Diseases Patients' Associations (EFA); European Federation of Neurological Associations (EFNA); European Heart Network (EHN); 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); Patients Network for Medical Research and Health (EGAN) **HCPWP members**: European Academy of Paediatrics (EAP); European Aids Clinical Society (EACS); European Association for Clinical Pharmacology and Therapeutics (EACPT); European Association of Hospital Pharmacists (EAHP); European Association of Urology (EAU); European Federation of Internal Medicines (EFIM); European Society for Medical Oncology (ESMO); European Society of Cardiology (ESC); Pharmaceutical Group of the European Union (PGEU); Standing Committee of European Doctors (CPME); The European Specialists Nurses Organisations (ESNO); United European Gastroenterology (UEG) **European Commission** (via videoconference) **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)

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Role	Name
	Observers: EMA Management Board; Co-ordination Group for Mutual Recognition & Decentralised Procedures – Human (CMD(h)); Patients' and Consumers' Working Party (PCWP); Healthcare Professionals' Working Party (HCPWP); Spanish Agency of Medicines and Medical Devices (AEMPS); European Academy of Allergy and Clinical Immunology (EAACI); European Forum for Primary Care (EFPC); European Society of Oncology Pharmacy (ESOP); Myeloma Patients Europe (MPE); Pain alliance Europe (PAE)

Introduction

I. Moulon (EMA) welcomed all participants and briefly introduced the topics to be covered throughout the day.

No conflicts of interests were disclosed in relation to the agenda items and the agenda was adopted.

1. EMA 20th Anniversary

1.1. Roadmap to 2020

N. Wathion (EMA) provided a high level overview of the draft EU Network Strategy to 2020. He explained that the document was developed in closed collaboration between the European Medicines Agency (EMA) and the Heads of Medicines Agencies (HMA) and is presented, for the first time, as a single strategy for the entire network to reflect the need for a coordinated approach to address the multiple challenges and opportunities that face the network.

The draft strategy focuses on areas where collaboration within the network can make a real difference to human and animal health in the EU over the five years to 2020. It builds on the EMA roadmap to 2015 and the HMA strategy document 2011-15.

The draft network strategy is arranged under four key themes focusing on: human health; animal health and human health in relation to veterinary medicines; optimising the operation of the network; and the global regulatory environment.

Separate multi-annual work programmes and implementation plans for EMA, HMA, and the Coordination Groups for Mutual Recognition and Decentralised Procedures, Human and Veterinary (CMDh and CMDv) will be developed. These will give detailed information on the work of each component of the network and describe how the strategy will be taken forward.

N. Wathion clarified that the Agency's multi-annual work programme could be of 3 or 5 years but a decision was still to be made. The work programme will be drafted with coordinated input from scientific committees and working parties and is expected to be adopted in December 2015.

PCWP and HCPWP members will be kept updated on progress and opportunities to provide input and this topic will be revisited at the next joint meeting in June, in the context of the 3-month public consultation on the draft network strategy.

1.2. Planned activities to mark the Agency's 20th anniversary

M-A. Heine (EMA) presented the activities planned to mark the 20th anniversary of the establishment of the European Medicines Agency (EMA) in 2015, which coincides with the 50th anniversary of the

introduction of the first EU legislation on human medicines (Council Directive 65/65/EEC). She outlined the main landmarks of the past 20 years underlining the increasingly important role of patients and healthcare professionals in the assessment of the risks and benefits of medicines and the fact that, meanwhile, patient representatives take part in most of EMA's scientific committees as full members, adding their unique perspective and experiences to the discussions.

Throughout 2015 several activities are scheduled (see presentation), the highlights being the EMA conference entitled 'Science, Medicines, Health: Patients at the heart of future innovation' and the publication of a 20th anniversary book, which captures the progress made in regulatory science and the changes in medicines regulation over the last 20 years, and describes the Agency's role in driving these changes.

The 20th Anniversary calendar includes many topics to be addressed in internal monthly events and PCWP/HCPWP members expressed an interest to be kept informed. Members supported the publication of brief videos and presentations on the topics to be covered. Particular areas of interest include health literacy, falsified medicines and initiatives to accelerate patients' access to innovative medicines.

2. Planning and reporting

2.1. Overview of EMA interaction with healthcare professionals during 2014

I. Silva (EMA) summarised the main achievements of 2014 (see presentation), which included a fully operational working party, an expanded network of eligible healthcare professional organisations, increased transparency, sustained involvement in core EMA activities, continued support to established EMA groups and networks, involvement in new initiatives and projects and continued efforts to raise awareness about the role of the EU Regulatory Network amongst healthcare professional organisations. The different activities will be further detailed in the Annual report on EMA's interaction with patients, consumers, healthcare professionals and their organisations, which will be published later in the year.

One participant raised the need to look into aspects of how to better communicate to healthcare professionals during the evaluation procedure (e.g. between different stages of a safety referral).

2.2. Patients' and healthcare professionals' feedback to EMA: survey results

M. Stroucken (EMA) gave an outline of the surveys carried out in January 2015 to father feedback from patients', consumers' and healthcare professionals' representatives on their involvement in EMA activities throughout 2014. Results show that overall levels of satisfaction are high, with some well identified areas for further improvement. The outcome of the surveys will be published as an annex to the Annual report on EMA's interaction with patients, consumers, healthcare professionals and their organisations.

One participant expressed a wish to see topics discussed at the HCPWP and PCWP meetings and/or as part of written consultations more contextualised in the flow of EMA activities and European initiatives. Another participant remarked the need to look into the EMA public calls for submission of data (e.g. safety referrals) to monitor whether input from patients was being submitted or not. He underlined that feedback received from some patients pointed to questions being difficult to respond and thus precluding capturing patient input.

2.3. Working methods for 2015: working groups, topic leaders, PCO/HCPO information sharing

N. Bere (EMA) presented a proposal to stimulate participation outside Working Party plenary meetings and promote further discussion of specific topics as well as to allow for a better utilisation of time allocated to face to face meetings. A number of topic groups were suggested to brainstorm in smaller groups between plenary meetings (see presentation).

The proposal was welcomed by PCWP and HCPWP members and an invitation to express their interests in the topic groups was launched together with a call for any other topics to be suggested.

2.4. Ebola update

M. Cavaleri (EMA) provided an update on the ongoing efforts to control the Ebola outbreak that began in March 2014 in West Africa, particularly summarising the Agency's activities as part of the global response against this outbreak (see presentation).

The Agency is working together with regulatory authorities around the world to support the World Health Organization (WHO) and to advise on possible pathways for the development, evaluation and approval of medicines to fight Ebola. In Europe, the Agency is supporting the work of the European Commission to facilitate information exchange between European Union (EU) Member States and to coordinate approaches on prevention of and preparation for Ebola outbreaks.

To support this work, the Agency has established a group of European experts who have specialised knowledge in vaccines, infectious diseases and clinical trial design. This group also gives advice to individual developers of Ebola medicines on scientific and regulatory matters. EMA is in close contact with developers of treatments and vaccines against Ebola, actively encouraging development of medicines and vaccines through programmes such as scientific advice or orphan designation.

In addition, the Agency maintains a continuous and open dialogue with international partners, mainly FDA and Health Canada, as well as with NGOS and scientists in charge of clinical trials in Africa. This level of communication is critical in the context of the progression of the Ebola outbreak and necessary to identify areas where regulatory flexibility and adjustment to approaches used for development and approval of medicines and vaccines may be needed.

3. EMA/EU-wide initiatives

3.1. Encouraging paediatric clinical research in the EU – current challenges

The chair introduced the topic and the discussion objectives, which included the importance to raise the general awareness about paediatric research in Europe, to better inform the public about clinical trials (CT) in children and their benefits and risks, and ultimately to improve perception of paediatric clinical research.

Following presentations from F. Schmidt (EC) and B. Pelle (EMA) outlining the successes and challenges faced by clinical research in children as well as the ongoing/planned activities at the level of the European Network of Paediatric Research at the European Medicines Agency (Enpr-EMA) and proposed initiatives at national level, participants engaged in an open debate. The key points emerging from the discussion are summarised below:

- a number of comments revolved on the wrong perception that healthy children are recruited in clinical trials, which highlighted the need to raise awareness about how CT are conducted in

children (drug development for children can focus on modelling and simulation or extrapolation of data to avoid involvement of healthy children; important to distinguish between control group and healthy children). It was however highlighted that the purpose of the discussion was not on the recruitment of healthy children into clinical trials but rather on the recruitment of children with an identified condition for which the investigational medicinal product was tested in clinical trial;

- approaches to engage children in research will need to take into account the different age groups, with distinctive strategies for children and young people;
- approaches will also depend on whether research is directed to children with congenital and/or life threatening conditions (e.g. congenital heart failure), or with chronic diseases with long remission periods (e.g. Crohn's disease) or where testing in a specific paediatric population can be extrapolated from adults to paediatric age subsets;
- need to develop targeted initiatives towards paediatricians, nurses, pharmacists and also teachers;
- attention needs to be given not only to recruitment (which is perceived as the current focus) but also and most importantly to protocol design (e.g. with particular attention to obstacles to participate in CT when the condition is too debilitating; how the child's and parents routines will be affected, etc.) and trial endpoints;
- the way trials are conducted have repercussions on public opinion and acceptability to consent for children to participate in a clinical trial; many companies wait for Phase III to develop CT for children and there is a need to encourage drug developers to start thinking of CT design for children much earlier;
- user friendliness of consent forms (need for review by established young person's advisory groups, when this is possible).

There was general support from PCWP and HCPWP members to provide information on European and national initiatives they were aware of in their field of activity and to take this discussion further in the context of the planned PCWP topic group dedicated to involvement of young people and children in clinical research.

3.2. Feedback from Scientific Committees

D. O'Connor, from the Committee for Orphan Medicinal Products (COMP), provided an update (see presentation) underlining the establishment of a COMP ad hoc Significant Benefit working group. Significant benefit is one of the criteria for orphan designation. It is defined in Regulation (EC) No 141/2000 as a "*clinically relevant advantage or a major contribution to patient care*". The concept of significant benefit is unique to the EU orphan legislation. The working group is discussing more in depth aspects related with consistency in requirements for the level of evidence needed to support significant benefit and how to assess a major contribution to patient care. D. O'Connor also mentioned the work related with assessing orphan designation applications for treatment of Ebola virus disease.

S. Bager, from the Herbal Medicinal Products Committee (HMPC), feedback on the Committee's activities highlighting some examples where input from patients and healthcare professionals could provide additional information related to the use of these medicinal products in real life (see presentation), taking into account that these medicines are mostly available as OTC products.

K. Breen, from the Committee for Advanced Therapies (CAT), referred that the Committee was analysing and discussing the responses received following the public consultation on the reflection paper on classification of advanced therapy medicinal products. The aim of this reflection paper is to

provide guidance on the ATMP classification procedure, as well as on the interpretation of key concepts of the definition of gene therapy medicinal product, somatic cell therapy medicinal product, tissue engineered product, and combined advanced therapy medicinal product. The guidance reflects the experience gained in the application of the classification procedure. An update on the outcome of these discussions is expected by mid-year.

A. van der Zeijden, M. Greco and K. Myhr from the Pharmacovigilance Risk Assessment Committee (PRAC) noted the remarks made by members of PCWP and HCPWP in relation to communication between different steps of safety procedures and pointed out the importance of earlier involvement of patients and healthcare professionals in PRAC discussions.

3.3. Survey to national competent authorities on stakeholder involvement

M. Mavris (EMA) presented the proposed set of questions sent out as part of a survey to national competent authorities. The survey aims at gathering input on national initiatives related to stakeholder involvement. Working Party members were invited to comment on the questions prior to the survey being shared with the HMA working group of communication professionals.

3.4. PROTECT / IMI project update

X. Kurz (EMA) gave an update on the IMI project for Pharmacoepidemiological Research on Outcomes of Therapeutics (PROTECT), focusing on its main results and recommendations as well as on the implications from a methodological perspective and for the evaluation of the benefits and risks of medicines, as discussed at the final project symposium organised in February 2015 (see presentation).

The methods developed by PROTECT focused on:

- enhancing early detection and assessment of adverse drug reactions from different data sources (clinical trials, spontaneous reporting and observational studies);
- establishing a framework for pharmacoepidemiology studies;
- examining new methods for data collection from consumers;
- exploring approaches to integrate benefit-risk methods into scientific assessment of medicines and the subsequent communication of these benefits and risks.

The Agency will now concentrate on translating the outputs of this project into outcomes for innovation and public health. The PROTECT project will be used as an example to test a conceptual framework for the review of the potential impact of outputs of regulatory science projects and the prioritisation of their implementation into regulatory practice and to make recommendations to EMA and its committees for an appropriate action on the project's results.

One participant suggested a reflection amongst patient organisations on how to find the high numbers of patients foreseen in the methodologies identified by the project and their logistical implications.

3.5. WEBRADR / IMI project update

S. Brosch (EMA) and V. Newbould (EMA) gave an overview of the IMI WEB-RADR project, highlighting its goals and current activities (see presentation). The project aims to explore the use of mobile technologies and social media to further improve the collection and analysis of information on suspected adverse drug reactions (ADRs). The project is looking at the use of mobile apps to report adverse reactions to National Competent Authorities, and the possibility identifying potential safety

issues with medicines from user comments (posts) on social media based on new visualisation technology and analytical methods. The project is also exploring how to utilise new technologies to best communicate safety information on medicines to patients, consumers and healthcare professionals.

F. Houyez (EURORDIS/PCWP) and A. Hadjipanayis (EAP/HCPWP) reported on the workshop that took place on 10 December 2014 (see presentations). The goal of the workshop was to provide a detailed understanding of the motivations for the project and the research areas, to stipulate the research aspects with the anticipated outcomes and to understand stakeholders' needs and concerns thus also addressing key areas of data privacy and ethical considerations.

4. EU Clinical trial regulation

4.1. EMA public consultation on application of transparency rules of EU Clinical Trial Regulation

F. Sweeney (EMA) presented the ongoing activities related with the implementation of the new clinical trial regulation and provided a more detailed insight on comments received in the context of the public consultation on the EMA draft proposal for an addendum, on transparency, to the "Functional specifications for the EU portal and EU database to be audited".

The key instrument to ensure transparency of clinical trials is the new clinical trial portal and database that will be used for submission and maintenance of clinical trial applications and authorisations within the European Union. The database will serve as the source of public information on clinical trial applications assessed, and clinical trials conducted in the EU, from the time of decision to authorise a trial up to the finalisation of those trials and inclusion of their results in the database. The Regulation gives EMA responsibility for its development and maintenance.

The aim of the public consultation was to seek stakeholders' views on the application of exceptions in relation to the transparency provisions of the European Clinical Trial Regulation, so that they strike the right balance between respecting patients' and doctors' needs and the publics' entitlement to extensive and timely information about clinical trials and developers' and researchers' need to protect their investments.

One participant remarked on the importance of the search functionalities and user friendliness of the interfaces for data presentation.

PCWP and HCPWP members will be kept updated on progress related to transparency rules and usability aspects of the portal and database interfaces.

4.2. EPF statement on "lay summary of clinical trial results"

EPF presented their statement on "lay summary of clinical trial results", which can be found in the link below: http://www.eu-patient.eu/globalassets/policy/clinicaltrials/epf-lay-summary-position-march-2015.pdf

5. A.O.B

There was no other business.

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

Close of meeting

Next meetings: 3 June 2015: PCWP meeting, 4 June 2015 (am): Joint PCWP/HCPWP meeting, 4 June 2015 (pm): HCPWP meeting