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Patient Health Protection

The role of patients[1] as members of the EMA Human Scientific Committees

The purpose of this paper is to define the role of patients within the different scientific committees of the EMA, focusing in particular on their role as members of such committees. It gives guidance to those patient representatives who are looking for nomination as member of a committee at the EMA; in this respect it addresses not only the work of the patients when they attend the committee but also the work involved outside of the committee and other related aspects.

This paper has been prepared in collaboration with patients' representatives who have been involved and have been members of the Human EMA scientific committees, based on their experience to date.

Community legislation (Regulation (EC) Nº 726/2004, Regulation (EC) Nº 141/2000, Regulation (EC) Nº 1901/2006, Regulation (EC) Nº 1394/2007, Regulation (EU) Nº 1235/2010 amending Regulation (EC) Nº 726/2004 and Directive 2004/27/EC) provides the basis for the participation and membership of patients in some of the EMA scientific committees while the "Framework on the Interaction between the EMA and Patients' and Consumers' Organisations" (EMEA/354515/2005-Final) establishes further the interaction between patients and consumers in these committees.

Recent analysis of the experience acquired so far[2] demonstrate that participation of patients in the scientific committees leads to increased transparency and trust in regulatory processes and develops mutual respect between regulators and the community of patients. It is also acknowledged that their contribution enriches the quality of the opinion given by the scientific committees. This positive experience confirms the importance for the Agency to continue supporting and facilitating patient contribution to the work of the committees.

Patients' representatives are additionally members of the EMA Management Board as stated in Article 65 (1) of Regulation (EC) Nº 726/2004, since September 2005.

This paper should be read in connection with:

- All related mandate and rules of procedures of each EMA scientific committee;
- Framework of interaction between the European medicines Agency and patients' and consumers' organisations (EMEA/354515/2005-Final);

[1] The term 'patient' is used throughout this document to refer to any individual who has been appointed member of an EMA scientific committee to represent the views and interests of patients in such committee.

[2] http://www.ema.europa.eu/docs/en_GB/document_library/Other/2010/01/WC500038080.pdf

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- Reflection paper on the further involvement of patients and consumers in the Agency's activities (EMEA/10723/2009);
- Rules for involvement of member of patients, consumers' and healthcare professionals' organisations in committee related activities (EMEA/483439/2008 rev. 1).

1. Patients as members of the EMA scientific committees

To date the implementation of community legislation has resulted in patients being members in three of the EMA scientific committees: the Committee for Orphan Medicinal Products (COMP), the Paediatric Committee (PDCO) and the Committee for Advanced Therapies (CAT). As of July 2012, patients will also be members of the Pharmacovigilance and Risk Assessment Committee (PRAC).

Committee for Orphan Medicinal Products (COMP)

Article 4 (3) of Regulation (EC) Nº 141/2000

The COMP includes in its membership three members nominated by the European Commission representing patients' organisations since its first meeting in April 2000, for a renewable term of three years.

Paediatric Committee (PDCO)

Article 4 (1.d) of Regulation (EC) Nº 1901/2006

The PDCO includes in its membership three members and three alternates appointed by the European Commission representing patients associations since September 2008, for a renewable term of three years.

Committee for Advanced Therapies (CAT)

Article 21 (1.d) of Regulation (EC) Nº 1394/2007

The CAT includes in its membership two members and two alternates appointed by the European Commission representing patients associations since January 2009, for a renewable term of three years.

Pharmacovigilance and Risk Assessment Committee (PRAC)

Article 61a(d) of Regulation (EU) Nº 1235/2010 amending Regulation (EC) Nº 726/2004.

Following a pilot phase, patients attend the meetings of the pharmacovigilance Working Party as observers. Once the new pharmacovigilance legislation comes into force as of July 2012, this working party will become a scientific committee and will include patients' representatives among its members.

Legislation does not currently include provisions for patients as members in the other two human scientific committees: the Committee for Medicinal Products for Human Use (CHMP) and the Committee on Herbal Medicinal Products (HMPC). However article 78 of Regulation (EC) Nº 726/2004 gives grounds for the involvement and ad-hoc consultation of patients in these two committees (usually on product-related issues).

Role and added value of patients as members of scientific committees

Patients who are members of EMA scientific committees act in the same way as all other members.

Patient's main role however is not expected to be of a scientific nature. Although experience has demonstrated that they very often contribute scientifically into the discussion, the added value of having patients and consumers in the scientific committees is to bring a unique and critical input based on their real-life experience of being affected by a disease and its current therapeutic environment. This element fills a gap which other committee members (so-called scientific experts) cannot fill, and which has proven necessary to achieve the best possible results within the regulatory process.

The role of patients in the scientific committees will therefore be the same as of any other member; however the patient/consumer's efforts should focus in ensuring that this unique perspective which only patients can bring (based on real-life experience as end users) is delivered throughout the committee's activities and outcome.

More in detail this can be:

- Reflecting on real-life implications of regulatory decisions.
- Helping and assisting in decision making so that the best decision is taken.
- Increasing transparency and building confidence and trust in the regulatory process.
- Ensuring credibility by guarantying that scientific regulatory bodies act for the benefit of society.
- Continuously contribute and ask for changes in the system to improve reliability.
- Representing patients' interests and providing a "patient perspective" view, on behalf of those directly affected by regulatory decisions.
- Bringing experience of the disease and/or identifying patients with experience of the disease that can be consulted when necessary.
- Reflecting about the risk that patients are prepared to take (or identify the adequate patients to ensure this aspect is considered by the committee).
- Identifying potential topics which may require or benefit from additional patient consultation.
- Actively contributing to patient information and communication related to medicines. Ensure that patients and patient's organisations can access useful and understandable information.
- Disseminating committees' outcomes when they become public; passing on information to other patients and patients' organisations.
- Bringing specific expertise from a patient communication-perspective (e.g. to put safety issues into context), including contribution to the decision on when to communicate.
- Ensuring that information in any document prepared by the committee for patients and the general public is clear and understandable by the target audience and that it fulfils their needs in terms of information content (e.g. wording of package leaflet, Q&As, etc).
- Advising and supporting regulators on the feasibility of planned investigations (e.g. for paediatric investigation plans (PIP), orphan designation, risk management plan, etc).
- Guarantee that scientific opinions address patient needs and that there is a rational and adequate use of incentives (e.g. in orphan designation) for the benefit of patients.

- Advising and supporting regulators in its dialogue with industry and other stakeholders when identifying areas of medical need for target research.
- Contributing, in a general capacity, to public health (raising awareness, where appropriate, of the impact of regulatory decisions) in the context of their organisation.

Members participate in accordance with the committee's rules of procedure and defined tasks. They must maintain confidentiality, declare any conflict of interest and abide by the EMA code of conduct.

Members take part in committee decisions and have equal voting capacity. Members should attend all the meetings and actively contribute to the discussions and to the work of the committee and where necessary, have awareness of specific therapeutic progress in specific areas.

Alternates may attend all committee meetings and contribute to the work and discussions within the committee. They may represent and vote on behalf of the nominated member when he/she is not in attendance at the meeting. Similarly, at the request of the member, the alternate may respond on behalf of the member in case of written procedures or any request for advice outside of meetings.

2. Other ways of participation of patients in scientific committees

In addition to being a member, patients can participate in three other different ways within the scientific committees:

- **Observers**, who may attend the whole meeting, participate in discussions, express their views but do not take part in committee conclusions or decisions.
- Observers participate in accordance with each committee's rules of procedure and must maintain confidentiality, declare any conflict of interest and abide by the EMA code of conduct.
- **Experts**, who advise the committee on specific issues and are selected for their relevant expertise, experience or knowledge; they bring a real-life experience of the disease and its current therapeutic environment. They act on their own behalf.
- Experts usually only attend part of a meeting to answer specific questions raised by the committee and do not take part in committee conclusions or decisions. They must maintain confidentiality, declare any conflict of interest and abide by the EMA code of conduct.
- In accordance with article 62(2) of Regulation (EC) No 726/2004, when involved as an expert, the patient is entered into the EMA EU expert database.
- **Representatives of a specific organisation**, who, on behalf of an organisation, usually attend part of a committee meeting to express the views of the organisation on a specific issue. They have the responsibility to liaise with their organisation to present the views of the organisation on the questions to be addressed.
- Representatives of organisations are not bound by confidentiality and the agreement of the applicant/sponsor/MAH should be sought prior to disclosure of any confidential data.
- Representatives are still expected to declare any conflict of interest and the organisations involved should be fully transparent with regard to their activities and funding sources.

Independently of the way in which patients participate in the scientific committees (i.e. members, experts, observers or representatives), they can bring four different features (not mutually exclusive):

Expertise	Convey a combination of specific education, training or professional experience
Experience	Convey practical disease knowledge obtained from direct contact with the disease (affected person or close contact with affected person, e.g. family, carer)
Advocacy	Act on behalf of the affected patients in defence of their rights; provide patient-oriented public health / healthcare policy perspective
Empowerment	Participate in decision-making process within the committee; having access to information and process on behalf of patients

Having a patient as member of the scientific committee does not guarantee systematically the necessary input for every discussion in terms of experience and expertise (depending on the therapeutic area or condition being discussed). Analysis of experience indicates that the best results are obtained by, in addition to the member, having ad-hoc involvement of the most adequate experts/representatives coming from the adequate patient's organisation in the field wherever necessary.

When involving experts and representatives of organisations the 'Rules of involvement of members of patients' / consumers' and healthcare professionals' organisations in committees related activities'[3] (EMEA/483439/2008 rev. 1) should apply.

3. Challenges, workload and conditions of patients as members of the EMA scientific committees

Based on their experience to date, patients' representatives who are members in EMA scientific committees have identified the following challenges they encounter and have also provided an indication of the implications of membership in terms of workload.

3.1 Challenges

- These are scientific committees and the discussion is mostly scientific (as well as regulatory) hence adequate preparation is necessary. The technicality of many of the discussions suggests that patients/consumers with some background on medicines regulation may find participation less demanding. The Agency, when possible, and depending on the specific circumstances (e.g. experience, complexity of topic) may provide support.
- It can be challenging to try and become familiar with many disease areas in order to follow the discussions surrounding the different therapeutic subjects.
- The pre-mail documentation sent in advance of the meeting can be difficult to manage in the beginning; however participants learnt to select those topics which would be of more interest to them.

[3] http://www.ema.europa.eu/docs/en_GB/document_library/Regulatory_and_procedural_guideline/2010/02/WC500074644.pdf

- Patient representatives often do not have the same resources as other delegates, both in terms of available time and access to information.
- The general workload of some meetings does not always allow time to advocate a patient perspective.

3.2 Workload

- Usually membership of an EMA scientific committee is for a period of 3 years, which may be renewed.
- The duration of meetings is as follows:
  - COMP – 2 to 3 day meeting per month (11 meetings per year).
  - PDCO – 3 day meeting per month (12 meetings per year).
  - CAT – 2 day meeting per month (11 meetings per year).
  - PhVWP – 3 day meeting per month (11 meetings per year).
- In terms of meeting preparation:
  - 4 days per month preparation and follow-up work outside of each meeting is felt necessary to guarantee an adequate contribution to the scientific committee; this fact should be clearly considered by any patients' organisation when proposing a member of an EMA scientific committee.
  - Time should be allocated to share, discuss and disseminate 'non-confidential' information within the organisation and to other stakeholders. This may imply additional work for other members/colleagues (patients) within a patient organisation.
  - The meeting attendance and work can be shared between the member and the alternate (with good communication to avoid discontinuity).

3.3 Conditions of participation

- Committee meetings are conducted in English. It is not possible to provide any translation service; therefore the patient representative should be fluent enough to be able to follow and participate to the meetings discussions.
- Meetings are held on week days and are not usually held during August (except PDCO).
- Travel and accommodation costs are covered by the EMA for each member, who will also receive a daily expenses allowance (extra financial support may be also provided in certain conditions).
- Although alternates are welcome to attend every meeting, they will only be reimbursed when the member is unable to attend.