European Medicines Agency

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Reflection Paper on the Further Involvement of Patients and Consumers in the Agency's Activities

Background note

In 2005 the Management Board endorsed a specific framework in the field of interaction between the Agency and Patients' and Consumers' Organisations. Two reports on the progress of the interaction have been published. As agreed by the Board in 2008 the Agency has actively worked together with Patients' and Consumers' Organisations on how to further develop the interaction in a more structured way. This resulted in the attached Reflection Paper.

Matters for consideration

In defining how to make progress on a more structured involvement of patients/consumers in the various activities of the Agency, two aspects have been considered:

- The role that patients/consumers are to play in the Scientific Committees, including their involvement in benefit/risk considerations.
- What kind of financial support should be provided to support patients/consumers when participating in the Agency's activities.

As can be noted from the attached Reflection Paper, the following proposals for action are made:

- To revise the current framework of interaction between the Agency and Patients' and Consumers' Organisations, including
  - defining the role of patients and consumers in the different Scientific Committees where they participate as members;
  - developing clear criteria on which situations need the consultation of and/or dialogue with patients' and consumers' representatives;
  - developing procedures in the areas of assessment of benefit/risk and preparation/provision of information to the public (especially on safety related aspects).
- To provide financial support by doubling the daily allowance in the following cases:
  - the delegate/expert must be a Management Board / Scientific Committee member appointed by the European Institutions, or and expert nominated by the Agency;
  - the delegate/expert must work on a voluntary basis for the organisation he/she represents and must not receive any other financial income for his/her work at the Agency;
  - the special allowance will be paid on the basis of each meeting day attended.

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REFLECTION PAPER ON THE FURTHER INVOLVEMENT OF PATIENTS AND CONSUMERS IN THE AGENCY'S ACTIVITIES

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Executive Summary

At the time of presenting the first report on the progress of the interaction with patients' and consumers' organisations (PCOs) to the Management Board in March 2008, it was agreed that the European Medicines Agency (hereafter referred to as the Agency) would actively work together with the PCOs who collaborate with the Agency, to explore how to further develop the interaction in a more structured way, and in this respect, it was decided that a Reflection Paper with proposed actions would be developed.

This Reflection Paper consists of three sections:

The first section elaborates on the best way to progress with the interaction of patients and consumers at the Agency. It builds upon the analysis of the experience achieved so far in involving patients and consumers (Section 2) as well as upon the results of the analysis of interest and capabilities for future involvement (Section 3). It is concluded that the current framework of interaction has been implemented, in particular those activities with specific legal basis (i.e. Management Board, COMP, PDCO and CAT). However, in other areas where no specific legal basis exists, patient interaction is not well structured, in particular those issues related to benefit/risk considerations (at the level of the CHMP).

Prior to the formulation of a proposal on how best to progress with the interaction and on how best to achieve a more structured involvement in the various Agency activities, some important considerations have been examined:

- The added value of patients and consumers in the scientific process of benefit/risk evaluation is confirmed, as they enrich regulatory outcomes by complementing them with the views of those directly affected by regulatory decisions. This is illustrated by various examples taken from existing experience at the Agency. A procedure to systematically assess the need to involve patients at different levels of the CHMP is proposed.

- The specific role that patients are to play in the different Scientific Committees and what is expected from their contribution should be defined. Initiatives in this area are ongoing: proposal for patient participation in meetings of the PhVWP following the outcome of the pilot phase and the current preparation of job descriptions for patients' representatives in the PDCO and the COMP.

- Considerations are also made on the need to financially support patients and consumers who participate in the Agency activities. It is acknowledged that they mostly work for their organisations on a voluntary basis and do not receive any financial support for the work they do at the Agency, nor any financial compensation for any loss of income or additional expenses incurred due to their attendance at the Agency. This is put into perspective with the current practice and experience at national level.

Having taken into account all previous analysis and considerations, the Board is asked to endorse the following proposals for action:

- Revision of the framework of interaction between the Agency and patients' and consumers' organisations;
- Provide financial support to experts and delegates in need of it under certain conditions.

The second section gives a thorough description of the different activities within the Agency in which patients and consumers have been involved so far, and analyses its added value, showing that patients and consumers bring a unique input which contributes to achieve the best possible results within the regulatory process. Their contribution not only builds trust in the mentioned process but also incurs higher levels of transparency. The main difficulties encountered concern the lack of resources and the enormous effort from the organisations in supporting their participation in the Agency activities. Training considerations to facilitate performance are also highlighted.

The third section explores the interest that PCOs have in progressing with the interaction, and aims at identifying priority areas based on their interest and on realistic expectations and capabilities in terms of resources from both PCOs and the Agency. For this purpose, a questionnaire was sent to all PCOs which fulfil the Agency's selection criteria for interaction. The results of the questionnaire confirm their interest, and also their request for the development of further involvement at CHMP level (e.g. consultation on product related issues, more participation in SAGs, PhVWP, SAWP, etc).

Introduction

Building upon the experience from various contacts with patients and consumers since the Agency's creation in 1995, the Management Board endorsed in 2005 a specific framework in the field of interaction between the Agency and patients' and consumers' organisations (EMEA/354515/2005-Final). This framework defined the objectives to be achieved in order to improve the structure and formalisation of this interaction.

Two reports on the progress of the interaction have been finalised. These reports showed that all actions identified in the framework of interaction had been implemented, and that the work achieved so far has established the grounds towards a more systematic interaction and involvement of patients and consumers at different levels of the Agency work.

At the time of presenting this report to the Management Board in March 2008, it was agreed that the Agency would actively work together with the patients' and consumers' organisations who collaborate with the Agency, to explore how to further develop the interaction in a more structured way, and in this respect it was decided that a Reflection Paper with proposed actions would be developed.

The objective of this Reflection Paper is:

To propose specific actions to the Management Board on how to further progress towards a more structured involvement of patients/consumers in the various Agency's activities. In order to achieve this, two fundamental questions need to be addressed:

- The role that patients and consumers are to play in the scientific committees, including to which extent patients should be involved in benefit/risk considerations.

- Whether the Agency should consider the provision of any kind of financial support to patients/consumers when participating in the Agency's activities.

The grounds for these proposals for action presented in Section 1 stem from the results of the analysis of two important aspects of patient/consumer interaction at the Agency:

- Description and analysis of the experience achieved so far in involving patients and consumers in the work of the Agency (Section 2).

- The interest that patients'/consumers' organisations have in becoming involved in certain defined activities of the Agency, which, "a priori" would benefit from patient input. This exploratory work considered realistic expectations and capabilities in terms of resources from both patients' and consumers' organisations and the Agency (Section 3).

Section 1
Patient and consumer involvement: the way forward

Analysis of the existing experience confirms that the "Framework of interaction between the Agency and patients' and consumers' organisations", endorsed by the Management Board in 2005 has been implemented.

Where articles in Community legislation foresee participation of patients as members of scientific committees (i.e. COMP, PDCO and CAT) this has been adequately implemented, and all related activities are well established. However, in other areas where no specific legal basis exists, despite positive feedback, patient interaction and involvement still occurs sporadically for many activities in particular those related to benefit/risk considerations (e.g., Ad-hoc contacts with CHMP and participation in SAGs).

The work already done gives the ground to plan and reflect on the way forward. The analysis of existing experience within the Agency (presented in detail in Section 2) together with the results of a questionnaire to patients' and consumers' organisations (presented in Section 3), confirms the interest from patients' and consumers' organisations in continuing to be involved in activities of the Agency.

Prior to formulating any proposals on how best to progress with the interaction and on how best to achieve a more structured involvement in the various Agency activities, some important considerations have been examined by the Agency:

1. Which would be the added value of consulting patients during the scientific process of benefit/risk evaluation and how should this interaction be established in practice?

2. What is the specific role that patients are to play in the different scientific committees, and what is expected from their contribution?

3. Consideration on financial support to patients/consumers.

1. Which would be the added value of consulting patients and consumers during the scientific process of benefit/risk evaluation?

In general, patients are expected to bring real-life experience of the disease and its current therapeutic environment; as a consequence:

- It would enrich regulatory outcome by complementing it with the views of those directly affected by regulatory decisions;
- It would increase confidence and trust in the regulatory process;
- It would lead to a higher level of transparency;
- It would allow to prepare for any future integration of patients as CHMP members when foreseen in new Community legislation.

To illustrate this, some examples in which patient contribution has been of added value to the scientific process (by reflecting on real-life implications of regulatory decisions) have been selected from the existing experience:

- feasibility of Risk Management Plan in real life environment (and feedback on its implementation);
- specific information to be put in the Package Leaflet and its wording;
- communication related issues (the decision on when to communicate, the content, etc);
- explore their views on the potential impact of a possible withdrawal or suspension;
- feasibility of CT design (e.g. availability of patients to enrol, acceptability of comparator);
- open dialogue with PCOs in certain situations (e.g. negative opinion). Explaining the reasons for such opinion, despite not being a consultation itself, can build transparency and trust in regulatory decisions, as it can lead to higher level of acceptability and understanding of regulatory decisions by medicine users.

Article 78 of Regulation (EC) Nº 726/2004 allows the CHMP and Rapporteurs appointed by the Committee to establish contacts on an advisory basis with representatives of patients' organisations (and healthcare professionals' associations) relevant to the indication of the medicinal product concerned. In accordance with this, a procedure should be developed so that benefit-risk assessments performed by the CHMP can take into account the view of patients and users of medicines when appropriate. In addition, this would result in full implementation of article 78 of Regulation (EC) Nº 726/2004.

Practical implementation of patient/consumer consultation during CHMP benefit/risk assessment should not be done systematically and only when potential added value is envisaged. Therefore, clear criteria should be developed in order to identify in a systematic and consistent way which cases need consultation and/or dialogue with patient's representatives. These criteria should be agreed by the CHMP and PCWP, and in all cases interaction should only be initiated after agreement by the CHMP.

Based on existing experience two levels of consultation are foreseen:

- As **representatives** of organisations (e.g. written consultation, and eventually meetings with (Co)-Rapporteurs on selected procedures -in the margin of the CHMP);

- As **experts** (e.g. in experts' meetings, SAGs meetings, review of PL and at the request of the (Co)-Rapporteur).

In both cases the "Rules of involvement of members of Patients'/Consumers' and Healthcare Professionals' Organisations in Committees related activities" (EMEA/483439/2008 Rev 1), will apply (see Annex 4).

In addition the following should be considered:

- Identification of patients' representatives will be done through the existing network of patients' organisations within the Agency, giving preference to those organisations which fulfil the defined "selection criteria" (see Annex 2) and therefore are eligible for interaction.

- Success will rely on both the motivation and performance of patients groups and the CHMP.

- There is a need to improve internal coordination, in order to guarantee systematic and timely identification (through defined criteria) of those issues which could benefit from patient/consumer input as they arise.

2. Role of patients in the different scientific committees

The specific role that patients are to play in the different scientific committees and what is expected from their contribution should be defined. Initiatives in this area are ongoing, as is the case of patient participation in meetings of the PhVWP following the outcome of the pilot phase and the current preparation of job descriptions for patients' representatives in the PDCO and COMP.

3. Consideration on financial support to patients/consumers

When patients and consumers attend meetings at the Agency, the basis for reimbursement of expenses has been the same as for all other experts and delegates who regularly attend meetings at the Agency. It covers cost of travel and hotel expenses plus a daily allowance.

It should, however, be pointed out that specific considerations apply to patients and consumers when they contribute to the Agency's work compared to other delegates, i.e.:

- Patients and consumers who work with the Agency are usually not nominated by National Competent Authorities, but by the Agency.

- Patients and consumers mostly work for their organisation on a voluntary basis, and do not receive any financial support for the work they do at the Agency, nor any financial compensation for any loss of income or additional expenses incurred due to their attendance at the Agency.

After having looked at current experience and practice regarding financial support to patients and consumers at European and national level (see Annex 5), the following applies:

- No distinction is made between patients and other experts/delegates (i.e. healthcare professionals, scientists, etc) involved in the work of any European Regulatory Authority with regard to reimbursement/financial compensation.

- Despite the fact that no specific formal financial support is in place for the work performed, two concepts for compensation of experts/delegates exist: a) allocation of a higher daily allowance and b) compensation to experts and delegates for any loss of income or additional expenses incurred due to their attendance to an Agency meeting (i.e. business unattended, locum general practitioner, nanny to care for children, etc).

Taking into account all these considerations as well as the experience analysed in this document, the following is concluded:

- A lack of financial resources makes participation difficult for patients and consumers in the activities of the Agency, as participation very often implies a great effort both from the expert/delegate and from the organisation.

- Patients and consumers are nominated by the Agency and mostly work on a voluntary basis; therefore neither their work nor the time invested in the Agency activities that they engage are remunerated.

- Previous analysis shows that their contribution to the work of the Agency is of equal value as that from other delegates.

Proposals for action

Having taken into account all previous analysis and considerations, the Board is asked to agree on the following proposals for action:

A. Revision of the framework of interaction between the Agency and patients' and consumers' organisations

The revision will cover the following aspects:

- Definition of the role of patients and consumers in the different scientific committees within the Agency where specific legal basis for their membership exists (including outcome of the ongoing work at the PhVWP);
- To enhance the number of European organisations collaborating with the Agency
- To provide adequate training to patients and consumers to facilitate and optimise their contribution to the Agency.
- Develop clear criteria in order to identify in a systematic and consistent way which cases need the consultation of and/or dialogue with patients' and consumers' representatives.
- Further to Article 78 and recital (18) of Regulation (EC) Nº 726/2004 and based on the list of priority areas identified for more systematic involvement of patients and consumers, specific procedures will be developed in the following areas:
    - Assessment of benefit/risk to facilitate the consultation of patients and consumers at the request of the CHMP;
    - Preparation and provision of information intended to the public by the Agency (especially on safety communication) to foster their involvement.

B. Provide financial support to experts and delegates in need of it

It is proposed to double the daily allowance received by experts and delegates in the following cases:

- The delegate/expert must be a member of a scientific committee/the Management Board appointed by the European Institutions, or an expert nominated by the Agency.
- The special allowance will only be paid on the basis of each meeting day attended by the delegate/expert.
- The delegate/expert must work on a voluntary basis for the organisation he/she represents and must not receive any other financial income for his/her work at the Agency.

Section 2
Analysis of existing experience

A thorough description of the different Agency activities in which patients and consumers have been involved so far is presented in this section of the document. For each activity, an analysis of the type of contribution, its added value as well as details of any difficulties encountered is provided.

a) Activities with specific legal basis

AGENCY Management Board

Since 2005, two members of the Agency's Management Board are representatives of patients' organisations. The legal basis for their membership is found in article 65 (1) of Regulation (EC) Nº 726/2004:

> *"The Management Board shall consist of one representative of each Member State, two representatives of the Commission and two representatives of the European Parliament.*
>
> *In addition, two representatives of patients' organisations, one representative of doctors' organisations and one representative of veterinarians' organisations shall be appointed by the Council in consultation with the European Parliament on the basis of a list drawn up by the Commission which includes appreciably more names than there are posts to be filled."*

The two representatives of patients' organisations have been acting from the beginning as members of the Board. They have been fully integrated into the work and activities of the Board and have actively participated both in the discussions and the decisions taken, providing their views as users of medicines and as representatives of civil society.

Analysis of their participation after many years shows a very positive and valuable contribution; their presence often adds value to the discussions and enables the Management Board to see certain questions from a different perspective.

As they quickly understood the role of the Management Board, their participation has always been focused. It is worth noting that a member of the Management Board representing doctors has been elected as a vice-chair for two consecutive terms. This demonstrates that civil society representatives have integrated very smoothly into the dynamics of the group.

AGENCY Scientific Committees

So far, three scientific committees include patients in their membership:

- The COMP (as per article 4 (3) of Regulation (EC) Nº 141/2000 on orphan medicinal products) has 3 representatives of patients' organisations since its first meeting in April 2000.
- The PDCO (as per article 4 (1.d) of Regulation (EC) Nº 1901/2006 on medicinal products for paediatric use) welcomed three members and three alternates, representing patients' organisations since September 2008.

- The CAT (as per article 21 (1.d) of Regulation (EC) Nº 1394/2007 on advanced therapy medicinal products) welcomes *two members and two alternates* representing patients' organisations *since 2009.*

Members representing patients' organisations participate in the committees' activities in the same way as any other members. They declare any conflict of interest they may have, sign confidentiality undertaking and adhere to the Agency code of conduct.

- **The experience of the COMP**

The COMP is the first scientific committee of the Agency in which patients' representatives have participated as members, and has by far the greatest experience having patients in its membership, not only in terms of time, but also in terms of the degree of involvement and participation.

The COMP can be considered as a model of good integration of patients within their operation, where the added value of their contribution to the committee's outcome is without doubt.

Patients have participated and continue to participate in all activities of the committee like any other member of the committee. As of today, it can be accredited for the following activities:

- Coordination for designation/review of designation criteria;
- Participation in oral explanations with sponsors;
- Preparation of list of questions;
- Provision of advice for nomination of experts;
- Input in the preparation of PSOs (Public Summaries of Opinion) as well as being an active part in the initiative to put them in place;
- Collaboration in drafting guidelines;
- Participation in Ad-hoc Groups, Working Groups and Working Parties related to the committee;
- Input in the common application FDA/ European Medicines Agency;
- Other activities outside of the Agency (e.g. monographic meetings on orphan designation and significant benefit, organised by their organisations).

Patient contribution has enriched the quality of the opinion given by the committee. Their main value comes from the fact that they bring new elements to the discussions even though they sometimes also provide scientific input relevant to the discussion, In this respect, patients guarantee that the opinion expressed by the committee takes more into consideration the potential implications for patients in real life.

Patients have understood very well where their input is of more value and which other aspects of the committee's discussions are out of their area of expertise and knowledge; at the same time the committee has also learnt to identify when to seek the patient views.

Through their many years of experience in the COMP, patients have attained knowledge and experience of the global regulatory environment and they have also gained a good understanding on the role of the Agency and its limitations. As a consequence, their expectations and proposals have, over time, become more focused, hence building trust between their organisations and the Agency.

Confidentiality has been very well maintained in all cases where patients have been involved in the work of the COMP, and no concerns have ever been identified.

With regard to any difficulties encountered during these years of participation, no major issues have been identified. Training was necessary and was provided by the Agency secretariat to the patients. The lack of resources and the enormous efforts made by the organisations to support patient participation in the committee has always been highlighted. Although some scientific background is always preferred for a good performance in activities, experience shows that patients without much scientific knowledge are also able to provide a very positive contribution, as their participation remained focused and within their limits.

With regard to conflict of interest, and as for any other expert, good expertise very often implied higher risk of conflict of interest.

- **The experience of the PDCO**

The PDCO is the second scientific committee within the Agency in which representatives of civil society participate as members.

Patients in the PDCO are involved in the PDCO's procedures in the same way as other members. A document defining the role and responsibilities of members and alternates, rapporteur and peer reviewers, experts and observers (EMEA/537415/2008) has been prepared and does not make any specific differentiation for patient representatives. So far, patients have been involved in several applications mainly as peer reviewers, as a way to progressively introduce them in the work of the Committee, although they have also already been acting as rapporteurs.

Patients are expected to bring additional paediatric expertise and experience to the committee, and in particular, as already mentioned for the COMP, their contribution can be very useful in providing input on real life implications of some discussions and decisions taken by the committee.

It is concluded that more experience is necessary at the level of the PDCO to draw a more precise analysis of the value of their contribution and of any potential difficulties identified for their participation. Work is ongoing in drafting a job description for patients as either experts or members of the committee, which would also apply to other committees. Training needs have already been identified and training sessions are being designed and implemented at the moment. A lack of resources to support patient participation in the committee has also been identified as an important issue.

- **The experience of the CAT**

  The CAT is the third scientific committee in the Agency in which representatives of patients' associations participate as members.

  Patients as members and alternates in the CAT were appointed in 2008 and have already participated in the early meetings of the CAT in 2009. Further experience is necessary in order to be able to analyse their contribution to this committee.

b) Activities under article 78 of Regulation (EC) Nº 726/2004

Article 78 of Regulation (EC) Nº 726/2004 remains the main piece of legislation used to develop appropriate contacts with patients and consumers in many activities of the Agency.

> *"The Management Board shall, in agreement with the Commission, develop appropriate contacts between the Agency and the representatives of the industry, consumers and patients and the health professions. These contacts may include the participation of observers in certain aspects of the Agency's work, under conditions determined beforehand by the Management Board, in agreement with the Commission."*
>
> *"The committees referred to in Article 56(1) and any working parties and scientific advisory groups established in accordance with that Article, shall in general matters establish contacts, on an advisory basis, with parties concerned with the use of medicinal products, in particular patient organisations and health-care professionals' associations. Rapporteurs appointed by these committees may, on an advisory basis, establish contacts with representatives of patient organisations and health-care professionals' associations relevant to the indication of the medicinal product concerned."*

The human scientific committees referred to in Article 56(1) and referred to human medicines are: CHMP, COMP, PDCO, CAT and HMPC.

In addition recital (18) of Regulation (EC) Nº 726/2004 states the following:

> *"The structure and operation of the various bodies making up the Agency should be designed in such a way as to take into account the need constantly to renew scientific expertise, the need for cooperation between Community and national bodies, the need for adequate involvement of civil society, and the future enlargement of the European Union. The various bodies of the Agency should establish and develop appropriate contacts with the parties concerned, in particular representatives of patients and health-care professionals".*

Other AGENCY scientific committees

- **The experience of the CHMP**

  Current legislation does not provide for patients and consumers to be members of the CHMP. However, in accordance to article 78 (2) of Regulation (EC) Nº 726/2004 the following activities have taken place at the level of the CHMP:

  - Ad-hoc meetings on product related issues under evaluation

    Examples of this type of meetings include the "meeting of victims' and patients' organisations on Thalidomide" and the "Ad-hoc expert group meeting on Viracept". In the first case, the Agency organised a joint meeting with victims of Thalidomide and patients and obtained a very valuable input in the design and feasibility of the risk management plan (RMP) for this medicine as well as on the information to be provided in the Package Leaflet. This meeting was regarded as an important step in the Agency interaction with patients' and consumers' organisations, which helped patients and victims to understand regulatory decisions which directly concerned them. It is also a good example of how to identify scientific issues which can benefit from patient input (RMP).
    In the case of Viracept, patients participated in this meeting together with other experts; they mainly contributed to the communication strategy in relation to the recall of Viracept due to contamination with harmful substances. The main difficulty found in these kind of cases is to identify in time suitable "patients experts" able to contribute to the scientific debate. The ongoing development of a wider network of "patients experts" in different areas should help to identify experts in a shorter timeframe.

  - Written consultations to eligible patients' and consumers' organisations

    Recently, the CHMP has decided to ask for the views of patients and consumers on medicinal products under evaluation. A list of questions to be answered in written by selected patients' organisations fulfilling the Agency's criteria for interaction was adopted by the committee. The existing experience (Onsenal and Prezista) has explored the experience and views of the organisations on certain aspects of the current use of the medicines. This is to be taken into account by the CHMP on any opinion it may adopt.

  - Participation in SAG meetings

    There are a few examples of patient participation in SAGs meeting, e.g. Tysabri, where very positive feedback on the patient contribution was collected. The basis for their participation as experts is similar to what has been previously considered for Ad-hoc expert meetings, and the same difficulties described above also apply.

    The rules of procedure for each SAG allow for their participation as experts and also for the SAG to establish contacts, on an advisory basis, with parties concerned with the use of medicinal products, in particular patient organisations.

- Participation in SAWP

Patients have been involved in the provision of the Agency's scientific advice since 2004. Their participation has been limited so far to procedures for protocol assistance. Despite having very few cases, the experience is viewed as very positive and definitely of having added value to the final advice provided. There has been some discontinuation in the level of patient participation during this period, mainly due to resource constraints from the organisations providing experts. Despite this, during 2008, patients participated in 4 procedures.

Patients have been invited to the Working Party to give their views on specific issues, and have attended plenary sessions, as well as discussion meetings with the companies. They have brought real life experience of the disease and of its current treatment environment, and have contributed importantly with issues such as the feasibility of the proposed trial design.

The main difficulties related to their participation which have been identified are:

- Scientific advice is a short procedure which makes it difficult to identify well in advance the need to involve them and consequently;
- The difficulty to find suitable experts able to contribute to the procedure within a short timeframe;
- Financial constraints, the Agency not having a tool to compensate for any lost income.

- Participation in guideline preparation (GTWP)

Two cases exist so far of patients and consumers having participated as experts in the preparation of guidelines before they were released for external consultation. They are the "*Guideline for follow-up of patients who have been administered gene therapy medicinal products*" and the "*Guideline on clinical development of medicinal products for treatment of HIV infection*". Bound by confidentiality, patients and consumers were included in the mailing list for the written circulation of documents, and they also attended the meetings of the relevant Working Parties where the guidelines were being discussed.

The contribution was found very useful for the preparation of the document, and input from patients and consumers was in line with other expert's contributions.

Again, difficulties in identifying what kind of guidelines would benefit from patient input, and in finding suitable patient experts is highlighted.

During the period of public consultation, the Agency is putting in place procedures to actively disseminate guidelines to concerned patients' and consumers' organisations to facilitate their timely input.

- Participation in PhVWP

  A pilot phase in which two patients have attended three consecutive meetings of the PhVWP as observers has been conducted. The experience has been used to identify in a report the "pros and cons" of the planned interaction, and covering issues such as the role that patients/consumers are to play in the PhVWP, their expected contribution to the work of the Working Party, as well as other aspects related to confidentiality.

  The report has been used for the development of the general strategy on how to further involve patients/consumers in the Agency activities as proposed in this "Reflection Paper".

  The object of this participation is also to facilitate a smooth integration of patients' representatives as members in the future pharmacovigilance committee as foreseen in the Commission's proposals for new pharmaco-vigilance legislation.

- **The experience of the HMPC**

  There is no legal basis for participation of patients and consumers as members of the HMPC, and any interaction is established through provision in article 78 (2) of Regulation (EC) Nº 726/2004. The HMPC has nominated a member who regularly attends the meetings of the Patients' and Consumers' Working Party (PCWP) and maintains links between the two groups. The HMPC has consulted the PCWP on various issues, and in this respect, activities involving patients and consumers, aimed at improving the way the Agency communicates information on herbal medicines to the general public is currently ongoing.

AGENCY Patients' and Consumers' Working Party (PCWP)

The AGENCY/CHMP Working Group with Patients Organisations was created following the EMEA/CHMP Workshop for Patients' Organisations held on 31 May 2002. This group looked at further improvements to be achieved in the areas of product information, transparency and dissemination of information, pharmacovigilance and interaction with the CHMP. Building on the work of this group and following the endorsement by the Management Board in 2005 of the "Framework of interaction between the Agency and PCOs", the group opened the possibility for other scientific committees to benefit from this forum of interaction, and therefore the mandate of the group was extended to allow CHMP, COMP and HMPC activities, and later on the PDCO and the CAT.

The Human Scientific Committees' Working Party with Patients' and Consumers' Organisations (commonly named as Patients' and Consumers' Working Party (PCWP) first met on 8th December 2006. It includes members' representatives of patients' and consumers' organisations and members' representatives from the different human scientific committees within the Agency. The PCWP has continued to be a platform for interaction and discussion between the Agency and PCOs on topics of common interest. It has played an essential role in fostering the progress of the interaction, and has helped identify which areas of interaction should be given priority. The PCWP has also contributed to all activities that the Agency has put in place in order to provide information adapted to patients and to the general public.

The PCWP meets four times a year and also, once a year, the PCWP welcomes representatives from every organisation which fulfils the Agency's eligibility criteria. In addition, one meeting is held together with representatives of healthcare professionals' organisations. Various drafting groups are also organised to discuss specific issues (e.g. European Commission's legislative proposal on information to patients, Agency website reconstruction, etc).

The PCWP will be looking at enlarging its composition in order to cover other areas which are currently not represented. In 2010, following its current mandate, a new election of Co-chairs will take place.

Feedback from PCOs on the PCWP is very positive and recognises the important value of the group. Through this work, PCOs have progressively understood the legal framework in which the Agency operates, as well as the role of the Agency and its limitations, making the interaction more efficient, being based on realistic expectations.

PCOs in the PCWP are in a privileged position, and are aware of their responsibility to act as "multipliers" of the outcome of the Agency interaction with members of their organisation.

AGENCY Working Group on Third Country Clinical Trials

Further to the "strategy paper: Acceptance of clinical trials conducted in third countries, for evaluation in Marketing Authorisation Applications" (AGENCY/228067/2008), an Working Group on Third Country Clinical Trials has been established within the Agency. The Working Group includes in its composition representatives from PCWP, who have been integrated as any other members in the different topics within the Agency's Action Plan 2008-2011.

Activities in relation with provision of information to patients/general public

The Patients' and Consumers' Working Party (PCWP) recommended that feedback be sought from patients on the readability of information contained in package leaflets, EPAR summaries, public statements and similar materials intended for the public.

Since May 2007 a procedure has been in place to consult patients' and consumers' organisations (PCOs) during the preparation of the English version of Package Leaflets (PLs) and EPAR summaries. The purpose of the consultation and interaction between the Agency and PCOs is to ensure that the information is clear and understandable for the target audience, and that it fulfils their needs in terms of information content.

Organisations fulfilling the Agency's eligibility criteria have been invited to propose experts that, after consideration, are nominated by the Agency. These experts are bound by confidentiality, should declare any interests they have and adhere to the Agency's code of conduct, similarly to any other expert participating in the Agency activities.

The legal basis for this interaction is again found in article 78 and Recital (18) of Regulation (EC) Nº 726/2004.

The analysis of the experience so far demonstrates that the comments received are of high quality and useful in their majority, as proven by the rate of implementation (about 60% for the PL and between 40 to 50% in the case of EPAR summaries).

As many experts in this procedure are not always familiarised with the Agency and its activities, a training session has been organised every year. This session has served to introduce the procedure to the experts, as well as to give them feedback and analysis of the existing experience. No issue in relation to confidentiality has been highlighted. Some problems were identified with regard to the establishment of the Eudralink system for each expert, and some help was necessary to facilitate its use by some experts. All of these issues, however, were solved smoothly.

In general, it is concluded that their contribution increases the quality of the revised documents within the scope of this procedure, for which more details are given below:

- **Review of EPAR summaries**
  Since May 2007 patients and consumers have performed a review of the English version of the EPAR summary for newly authorised medicines at the time of its preparation by the Agency.

- **Review of Package Leaflet**
  Patients and consumers have performed a review of PLs at the time of the renewal of a marketing authorisation since May 2007, and from September 2008, the procedure has been extended to cover the review of PLs at the time of initial evaluation for marketing authorisation.

- **Involvement in safety communication**
  During the past few years, patients have been involved in selected cases in the preparation of "Product Safety Announcements". Patients have given their input during the preparation of "Questions and Answers" documents addressed to the general public. Patients participating in these pilot exercises were selected from the network of patient experts which has been created in relation to the review of documents, and who had experience in the specific therapeutic area related to the safety issue. They gave input on whether information was clear and understandable, but most importantly that the information addressed all issues that from a patient perspective need to be clarified.

  Some examples of this interaction are Viracept, Neupro and Exubera. For all of them the feedback has been very positive in providing a useful and well focused contribution. Difficulties identified mainly refer to a "*per se"* strict procedure in terms of timing and the difficulty to get suitable experts in a very short timeframe. Despite high sensitivity of the nature of some issues, no concern with regard to confidentiality has been identified. The need to further involve patients as experts when preparing product safety announcements has been already requested by the PCWP on various occasions, and their value is known by the Agency from the existing examples. The Agency will explore how to establish a more systematic approach. In addition it is necessary to set-up a system which guarantees that product safety announcements together with other critical issues communicated by the Agency reach those stakeholders concerned in a timely manner.

- **Involvement in other documents intended to the general public.**
  Patients and consumers have also participated in the preparation of other documents, such as the "Questions and answers on generics", the "Questions and answers on biosimilars" and the "Questions and answers on the regulation of advanced therapy medicinal products".

Other activities

- **Involvement in AGENCY transparency projects**

  Various Agency projects in relation to transparency have been subject to specific consultation with Agency's stakeholders, among which patients have always been included. Examples of these consultations are the new Transparency Policy, Eudrapharm, EudraCT, "Provision of information to stakeholders", reconstruction of the Agency's website, etc.

  The initiatives have been much appreciated and their value recognised by both the Agency and stakeholders, as it provides useful input on basic principles of complex projects at an early stage of development.

  The Agency's current trend is to consider these kinds of consultations more systematically for ongoing transparency projects. Patients and consumers participate representing their organisations and selection of organisations has never been an issue, as the eligibility criteria have facilitated this task. The PCWP has often collaborated, and has helped by making the consultation more efficient.

- **Participation in conferences/workshops organised by the Agency**

  The Agency has ensured that patients and consumers participate in conferences and workshops making valuable contribution. These events may be of scientific nature or can cover other general regulatory issues. Some examples are mentioned:

  - Workshop on "First-in-man clinical trials for potencial high-risk medicinal products guideline";
  - EC/EMEA Conference on the operation of the clinical trials directive;
  - workshop on SMA outcomes";
  - Workshop on naming, labelling and pack design of insulin containing medical products;
  - Workshop on EudraCT - Patient group consultation for public web site;
  - Workshop on user testing DIA-EMEA.

  As mentioned above, systematic consideration for patients and consumers participation is becoming the rule, and the criteria for interaction help to decide which organisations should be invited. Patients and consumers participate as representatives of their organisations and no confidential information is discussed during the conferences/workshops.

Conclusions

After analysing all existing experience, we can distinguish four different ways of participation of patients and consumers in the Agency's activities:

- Members (of MB, committees and working parties);
- Experts;
- Representatives of their organisations;
- Observers.

In all cases specific conditions apply (e.g. with regard to confidentiality undertaking and declaration of interests). These considerations together with a correct definition of the above mentioned four categories will be described in the "Rules of involvement of members of Patients'/Consumers' and Healthcare Professionals' Organisations in Committees related activities" (EMEA/483439/2008 Rev 1), which will be revised. (see Annex 4).

- **Analysis of benefits derived from patient involvement:**

  - Patients and consumers usually bring a unique input and a different perspective to those activities in which they participate. Usually they contribute by encouraging reflection about the real-life implications of regulatory decisions.

  - Their contribution is very often taken into account, is valid for the activity and improves the regulatory outcome.

  - Across the different activities of the Agency, patients and consumers have always proved to be very focused in their participation, as they have often identified those areas where their input could be of value, but also understood the limits of their participation. Consequently integration has been generally very smooth.

  - Patients and consumers have always contributed to the scientific committees in a responsible manner and in accordance with the spirit of Community legislation (e.g. they have not favoured products to obtain positive opinion unless clear benefits to the patients were demonstrated).

  - Patients participating as experts perform as any other expert who works with the Agency and therefore technically no distinction should be applied (e.g. they all sign declaration of interest and confidentiality undertaking and adhere to the Agency's code of conduct).

  - Confidentiality has been very well maintained in all cases where patients have been involved, and no concerns have ever been identified.

  - Through many years of experience of interaction with the Agency, patients and consumers have acquired knowledge and experience of the global regulatory environment, as well as the legal framework in which the Agency operates. As a consequence, their expectations and proposals have in time become more focused, hence building trust in the regulatory process.

  - Patient involvement incurs higher level of transparency.

- **Analysis of difficulties encountered in the involvement:**

  - Lack of resources and the enormous effort from the organisations in supporting patient/consumer participation in activities of the Agency is considered the main difficulty hindering interaction.

  - Training considerations (time that patients usually need to get familiarised with the procedures) is also highlighted. Some patients' organisations, in collaboration with the Agency are organising training sessions for patients to facilitate their involvement in regulatory activities.

  - Difficulties in defining the precise role that patients should have in a determined activity. In this aspect, it is important that the Agency specifically defines the role that patients are to play as members of the different scientific committees, clearly identifying what is expected from their contribution. Initiatives aiming to define the role of committee's members are ongoing (e.g. pilot phase of patient participation as observers in meetings of the PhVWP; preparation of job descriptions for patients representatives in the PDCO and COMP)

  - Difficulties in finding suitable patients experts able to contribute to a specific activity, usually in a short timeframe. In this respect, the language barrier needs to be pointed out, as a suitable patient expert should have a good command of English to be able to participate. The creation of a wider network of patients/consumers experts is expected to help mitigate this issue.

Section 3
Analysis of interest and capabilities for future involvement

Based on existing experience, further progress on the overall interaction should be planned building upon realistic interest expressed by patients' and consumers' organisations, as well as capabilities in terms of resources.

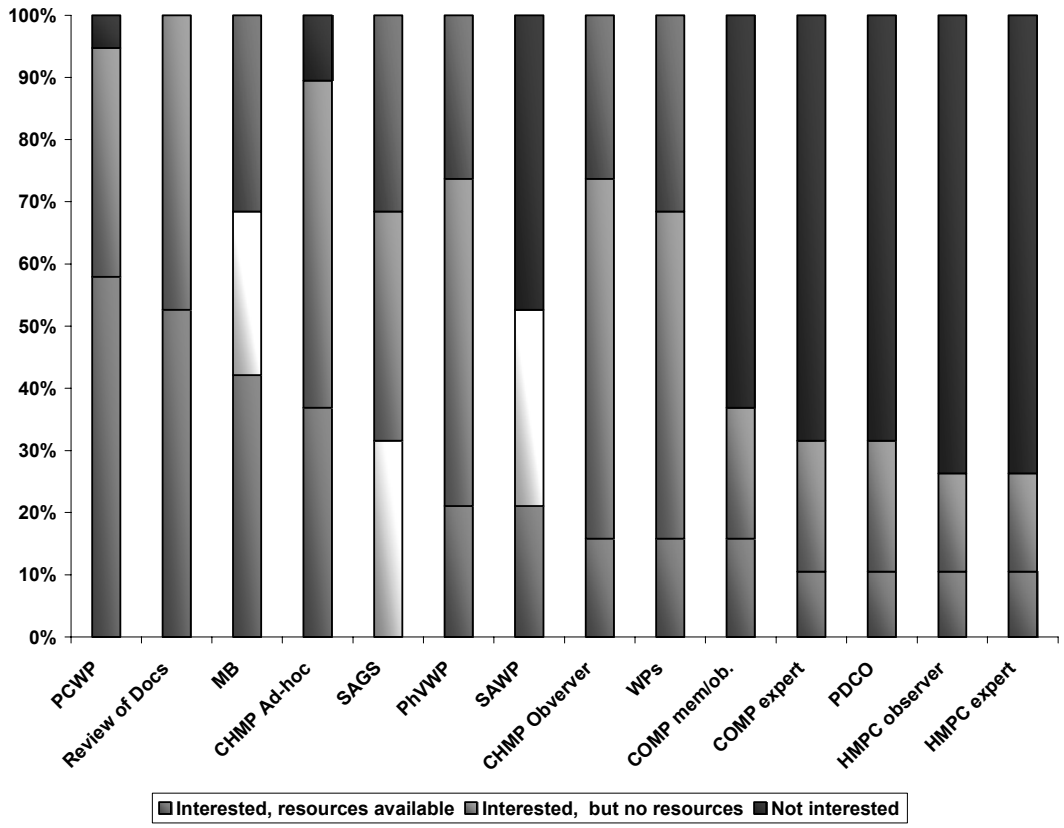
Exploratory work in this area would help the Agency to identify which areas or activities should be given priority for interaction. It would also help to identify the limitations in terms of resources that an organisation faces and which may prevent their participation in certain activities despite their interest.

In this respect a questionnaire was sent to 19 organisations which fulfil the Agency's selection criteria for interaction, and are therefore eligible to participate in the Agency activities. These organisations are more familiar with the Agency and its work, and consequently more likely to provide a valuable input. The questionnaire identifies several activities for which patients/consumer involvement would bring an added value (including those activities mandated by community legislation). Patients' organisations were asked about their interest to become involved in each of the identified activities, and if resources would limit their participation. The full questionnaire sent to the organisations is presented in Annex 3.

The following organisations were consulted and provided a valid response to the questionnaire:

1. Alzheimer Europe (AE)
2. European AIDS Treatment Group (EATG)
3. European Cancer Patient Coalition (ECPC)
4. European Federation of Neurological Associations (EFNA)
5. European Genetic Alliances' Network (EGAN)
6. European Multiple Sclerosis Platforms (EMSP)
7. European Myeloma Platform (EMP)
8. European Organisation for Rare Diseases (EURORDIS)
9. European Parkinson's Disease Association (EPDA)
10. European Patients' Forum (EPF)
11. European Public Health Alliance (EPHA)
12. Health Action International (HAI)
13. Insulin Dependent Diabetes Trust (IDDT)
14. International Alliance of Patients' Organizations (IAPO)
15. International Diabetes Federation (IDF)
16. Myeloma Euronet (ME)
17. Rett Syndrome Europe (RSE)
18. Thalaessaemia International Federation (TIF)
19. The European Consumers' Organisation (BEUC)

The following chart summarises the results of the questionnaire, and presents the different activities that patients' and consumers' organisations were questioned about. It measures de degree of interest declared for each activity considering they could allocate resources to it; it also identifies the degree of interest to participate in each activity but when no resources are available. Finally, it also indicates when no interest exits for an activity independently of resources.



The results of the questionnaire were discussed during the plenary PCWP meeting in December 2008, and patient and consumer representatives agreed that different constraints (e.g. human resources, training, etc) which may hinder performance when participating in the Agency activities, ultimately depends on the organisations' financial resources.

In addition during the PCWP discussion it was agreed that this information should be treated as a "trend" of patients/consumers interest, as patients and consumers representatives declared having answered the questions on the basis of their current involvement at the Agency, expressing where they would like to be further involved in the future. This may leave activities where patient involvement is already well established (e.g. scientific committees with patients as members) last in the priority list. Bearing in mind this fact, results of the questionnaire allowed for the preparation of the list of priority activities shown below. This order has to be considered when planning further progress in the interaction of the Agency with patients' and consumers' organisations.

- **List of priority activities for further involvement**

    - Management Board;
    - CHMP (ad-hoc consultation);
    - Scientific Advisory Groups;

- Pharmacovigilance Working Party;
- Scientific Advice Working Party;
- CHMP (observer);
- Committee on Orphan Medicinal Products;
- Working Parties (guidelines);
- Paediatric Committee;
- Committee on Herbal Medicinal Products.

Annexes

Annex 1

Legal basis for the interaction and involvement of patients and consumers

Provisions in relation to contacts between the Agency and patients' and consumers' organisations:

- Article 78 (1 and 2) of Regulation (EC) Nº 726/2004:

  "The Management Board shall, in agreement with the Commission, develop appropriate contacts between the Agency and the representatives of the industry, consumers and patients and the health professions. These contacts may include the participation of observers in certain aspects of the Agency's work, under conditions determined beforehand by the Management Board, in agreement with the Commission."

  "The committees referred to in Article 56(1) and any working parties and scientific advisory groups established in accordance with that Article, shall in general matters establish contacts, on an advisory basis, with parties concerned with the use of medicinal products, in particular patient organisations and health-care professionals' associations. Rapporteurs appointed by these committees may, on an advisory basis, establish contacts with representatives of patient organisations and health-care processionals' associations relevant to the indication of the medicinal product concerned."

- Recital (18) of Regulation (EC) Nº 726/2004:

  "The structure and operation of the various bodies making up the Agency should be designed in such a way as to take into account the need constantly to renew scientific expertise, the need for cooperation between Community and national bodies, the need for adequate involvement of civil society, and the future enlargement of the European Union. The various bodies of the Agency should establish and develop appropriate contacts with the parties concerned, in particular representatives of patients and health-care professionals."

- Article 65 (1) of Regulation (EC) Nº 726/2004:

  *"**The Management Board** shall consist of one representative of each Member State, two representatives of the Commission and two representatives of the European Parliament.*

  In addition, two representatives of patients' organisations, one representative of doctors' organisations and one representative of veterinarians' organisations shall be appointed by the Council in consultation with the European Parliament on the basis of a list drawn up by the Commission which includes appreciably more names than there are posts to be filled."

- Article 4 (3) of Regulation (EC) Nº 141/2000 on **orphan medicinal products**:

"The Committee shall consist of one member nominated by each Member State, three members nominated by the Commission to represent patients' organisations and three members nominated by the Commission on the basis of a recommendation from the Agency. The members of the Committee shall be appointed for a term of three years, which shall be renewable."

- Article 4 (1.d) of Regulation (EC) Nº 1901/2006 on medicinal products for **paediatric** use:

  "The Paediatric Committee shall be composed of the following members:
  (…)

  (d) three members and three alternates appointed by the Commission, on the basis of a public call for expressions of interest, after consulting the European Parliament, in order to represent patient associations".

- Article 21 (1.d) of Regulation (EC) Nº 1394/2007 on **advanced therapy medicinal products**:

  "The Committee for Advanced Therapies shall be composed of the following members:
  (…)

  (d) two members and two alternates appointed by the Commission, on the basis of a public call for expressions of interest and after consulting the European Parliament, in order to represent patients' associations."

Provisions in relation to the provision of quality information to patients and the general public:

- Publication of an **EPAR summary** understandable to the public (Article 13 (3) of Regulation (EC) Nº 726/2004):

  "The European Public Assessment Report (EPAR) shall include a summary written in a manner that is understandable to the public. The summary shall contain in particular a section relating to the conditions of use of the medicinal product."

- Publication of information on the **withdrawal** of an application (Article 11 of Regulation (EC) Nº 726/2004):

  "If an applicant withdraws an application for a marketing authorisation submitted to the Agency before an opinion has been given on the application, the applicant shall communicate its reasons for doing so to the Agency. The Agency shall make this information publicly accessible and shall publish the assessment report, if available, after deletion of all information of a commercially confidential nature".

- Publication of information on the **refusal** of a marketing authorisation (Article 12.3 of Regulation (EC) Nº 726/2004):

  "Information about all refusals and the reasons for them shall be made publicly accessible."

- Publication of information on **compassionate use** programmes (Article 83.6 of Regulation (EC) Nº 726/2004):

  "The Agency shall keep an up-to-date list of the opinions adopted in accordance with paragraph 4 (compassionate use), which shall be published on its website."

- Publication of **specific obligations** and conditional approvals (Article 14.7 of Regulation (EC) Nº 726/2004):

  "Following consultation with the applicant, an authorisation may be granted subject to certain specific obligations, to be reviewed annually by the Agency. The list of these obligations shall be made publicly accessible."

- **Assisting the Community and Member States** in the **provision of information** to the public on centrally authorised medicines (Article 57. 1 (m) of Regulation (EC) Nº 726/2004:

- *"(…) the Agency, acting particularly through its committees, shall undertake the following tasks:*

  (m) assisting the Community and Member States in the provision of information to health-care professionals and the general public about medicinal products evaluated by the Agency."

- Provision of appropriate **pharmacovigilance information** to the general public (Article 57. 1 (d, f) of Regulation (EC) Nº 726/2004):

  "(…) the Agency, acting particularly through its committees, shall undertake the following tasks:

  (d) ensuring the dissemination of information on adverse reactions to medicinal products authorised in the Community, by means of a database permanently accessible to all Member States; health-care professionals, marketing authorisation holders and the public shall have appropriate levels of access to these databases, with personal data protection being guaranteed;

  (f) distributing appropriate pharmacovigilance information to the general public."

- Development of an EU wide **database** on medical information on all medicinal products in the EU accessible and understandable by the general public (Articles 57.1 (l) and 57.2 of Regulation (EC) Nº 726/2004):

  "1. (…) the Agency, acting particularly through its committees, shall undertake the following tasks:

  (l) creating a database on medicinal products, to be accessible to the general public, and ensuring that it is updated, and managed independently of pharmaceutical companies; the database shall facilitate the search for information already authorised for package leaflets; it shall include a section on medicinal products authorised for the treatment of children; the information provided to the public shall be worded in an appropriate and comprehensible manner;

2. The database provided for in paragraph 1(l) shall include the summaries of product characteristics, the patient or user package leaflet and the information shown on the labelling. The database shall be developed in stages, priority being given to medicinal products authorised under this Regulation and those authorised under Chapter 4 of Title III of Directive 2001/83/EC and of Directive 2001/82/EC respectively. The database shall subsequently be extended to include any medicinal product placed on the market within the Community."

- Article 41 of Regulation (EC) Nº 1901/2006 in relation to **public information of paediatric clinical trials**:

  "By way of derogation from the provisions of Article 11 of Directive 2001/20/EC, the Agency shall make public part of the information on paediatric clinical trials entered in the European database."

- Recital (17) of Regulation (EC) Nº 1901/2006 on medicinal products for **paediatric** use:

  "To provide healthcare professionals and patients with information on the safe and effective use of medicinal products in the paediatric population and as a transparency measure, information on the results of studies in the paediatric population, as well as on the status of the paediatric investigation plans, waivers and deferrals, should be included in product information."

Annex 2

London, 7 February 2005
Doc. Ref. AGENCY/14610/04/Final
Approved by MB 29 September 2005

Criteria to be fulfilled by Patients' and Consumers' Organisations involved in the Agency Activities

I. Introduction

This paper has been developed to define the criteria patients' and/or consumers' organisations should fulfil in order to be involved in the Agency activities, such as the COMP or the CHMP/EMEA working group with patients and consumers' organisations.

These criteria do not apply to the procedure for external consultation on documents, since such external consultation is open to all external parties.

II. Definition of Patients'/Consumers' Organisations

Patients' organisations are defined as not-for profit organisations which are patient focused, and whereby patients and/or carers (the latter when patients are unable to represent themselves) represent a majority of members in governing bodies.

These could be:

- either general umbrella organisations (e.g. representing either European specific disease organisations and/or national umbrella organisations)
- or European disease specific organisations (i.e. representing national organisations or individual patients on acute and/or chronic diseases).

Consumers' Organisations are defined as not-for profit organisations which defend and promote the general interests of European consumers - citizens as purchasers or users of goods and services.

III. Criteria to be fulfilled

The organisations should be established at European Union (EU) level, and should fulfil the following criteria:

- Legitimacy: the organisation should have statutes registered in one of the Member States of the EU. If it is an international organisation not registered in a EU Member State, additional information needs to be provided demonstrating EU focus and activities.

- Mission/Objectives: the organisation should have its mission/objectives clearly defined and should agree to have it/them published on the website of the Agency.

- Activities: the organisation should have, as part of its activities, a specific interest in medicinal products which should be documented (e.g. through a report published on the organisation website).

- Representativity: the organisation should be representative of patients or consumers throughout the EU. Organisations already registered at Community level, e.g. in the EU Health Forum, the Council of Europe, are considered to adequately represent patients or consumers for involvement in the Agency activities.

- Structure: the organisation should have governing bodies which are elected by their members, who shall be patients, their carers, or their elected representatives.

- Accountability and Consultation Modalities: statements and opinions of the organisation should reflect the views and opinions of its members and adequate consultation procedures with those members should be in place. In particular, the organisation should ensure that the appropriate flow of information is in place to allow dialogue both ways: from and towards its members.

- Transparency: as a general rule, the organisation should be as transparent as possible, e.g. by regularly publishing, on its website, a report on the activities undertaken. The organisation should also disclose its sources of funding both public and private by providing the name of the public and/or private bodies and their individual financial contribution in terms of percentage of the organisation budget. Any relationship with corporate sponsorship should be clear and transparent. Any conflict of interest should be disclosed to the Agency. In case of umbrella organisations the list of member associations should be publicly available.
  The reference to private bodies does not include private individuals unless this presents a potential conflict of interest as referred to above.

In addition, Patients' and Consumers' Organisations shall be committed to take active part in the interaction with the Agency. To facilitate communication, a contact person shall be identified.
In case of lack of European associations for a specific disease or treatment areas, the involvement of national organisations may be considered even though preference will be given to European wide-associations. These associations will need to fulfil the same criteria apart from representativity which will be at national level.

In case of several associations existing in different Member States, a choice will be considered on a case-by case basis.

In order to further increase the transparency in this field, the Agency will create a public registry of those patients' and/or consumers' organisations with whom it will interact, as a consequence of the fulfilment of the above criteria.

Annex 3

QUESTIONNAIRE

Dear patient/consumer,

You have been asked to take part in this survey since you are part of an organisation eligible to participate in AGENCY activities.

Your answers to the following questions will help us understand in which activities your organisation would like to be involved at the Agency.

The questionnaire will also help us to know if your organisation has the necessary resources to commit to participate in such activities.

1. My organisation is:

2. My name is (optional):

Organisation

Name:

Email Address:

Phone Number:

Other (please specify):

Please tick from the boxes below the activities that your organisation would like to be involved in

1. CHMP* (Committe for Medicinal Products for Human Use):
- participation as observer in the committee plenary meetings

□ □□ Interested, and my org. has all the necessary resources
□ □□ Interested, but my org. does not have the necessary resources
□ □□ Not interested
Comments:

2. CHMP (Committee for Human Medicinal Products):
- participation as expert in ad-hoc discussions*

□ □□ Interested and my org. has all the necessary resources
□ □□ Interested, but my org. does not have the necessary resources
□ □□ Not interested
Comments:

3. PhVWP (Pharmacovigilance Working Party):
- participation as observer

□ □□ Interested and my org. has all the necessary resources
□ □□ Interested, but my org. does not have the necessary resources
□ □□ Not interested
Comments:

* The Committee for Medicinal Products for Human Use (CHMP) is responsible for preparing the Agency's opinions on all questions concerning medicinal products for human use

* During the CHMP meeting several ad-hoc discussions take place. Participation in ad-hoc discussions includes product related issues under evaluation by the committee (e.g. meetings with the rapporteur/co-rapporteurs or selected CHMP members in discussions out of the scope of the scientific advisory groups). The participation would look at obtaining the view of patients/consumers on issues such as risk minimisation measures, etc...

CHMP working parties have expertise in a particular scientific field. The CHMP consults its working parties according to their expertise, related to the evaluation of marketing authorisation applications or for drafting and reviewing scientific guidance documents. CHMP Working parties meet on a regular basis.

4. SAWP (Scientific Advice Working Party):
- participation as expert in selected procedures

☐ ☐☐ Interested and my org. has all the necessary resources
☐ ☐☐ Interested, but my org. does not have the necessary resources
☐ ☐☐ Not interested
Comments:

5. PCWP (Working Party with Patients' and Consumers' Organisations):
- participation as member

☐ ☐☐ Interested and my org. has all the necessary resources
☐ ☐☐ Interested, but my org. does not have the necessary resources
☐ ☐☐ Not interested
Comments:

6. Other working parties*:
- participation as expert

☐ ☐☐ Interested and my org. has all the necessary resources
☐ ☐☐ Interested, but my org. does not have the necessary resources
☐ ☐☐ Not interested
Comments:

* Other working parties in which you might be interested in participating as expert for activities such as: guidelines preparation, consultations on a case-by-case basis, are:
Biologics Working Party (BWP)
Blood Products Working Party (BPWP)
Cell-based Products Working Party (CPWP)
Efficacy Working Party (EWP)
Gene Therapy Working Party(GTWP)
Joint CHMP/CVMP Quality Working Party (QWP)
Phamacogenomics Working Party (PgWP)
Safety Working Party (SWP)
Vaccine Working Party (VWP

7. Scientific Advisory Groups*:
- participation as expert

* Scientific advisory groups (SAGs) are established by the CHMP to provide advice in connection with the evaluation of specific types of medicinal products or treatments. Their meetings are organised on a case by case basis upon request of the CHMP.
The role of patients would be to represent the view of patients/carers/users in the SAG meeting.

☐ ☐☐ Interested and my org. has all the necessary resources
☐ ☐☐ Interested, but my org. does not have the necessary resources
☐ ☐☐ Not interested
Comments:

Please tick from the boxes below the activities that your organisation would like to be involved in

1. AGENCY Management Board:
- participation as member

☐ ☐☐ Interested and my org. has all the necessary resources
☐ ☐☐ Interested, but my org. does not have the necessary resources
☐ ☐☐ Not interested
Comments:

2. COMP* (Committee for Orphan Medicinal Products):
- participation as member/observer

□ □□ Interested and my org. has all the necessary resources
□ □□ Interested, but my org. does not have the necessary resources
□ □□ Not interested
Comments:

3. COMP* (Committee for Orphan Medicinal Products):
- participation as expert in other activities including consultations on the information produced by the committee + ad-hoc discussions

□ □□ Interested and my org. has all the necessary resources
□ □□ Interested, but my org. does not have the necessary resources
□ □□ Not interested
Comments:

4. HMPC** (Committee for Herbal Medicinal Products):
- participation as observer

□ □□ Interested and my org. has all the necessary resources
□ □□ Interested, but my org. does not have the necessary resources
□ □□ Not interested
Comments:

* The Committee for Orphan Medicinal Products (COMP) is responsible for reviewing applications from persons or companies seeking 'orphan medicinal product designation' for products they intend to develop for the diagnosis, prevention or treatment of life threatening or very serious conditions that affect not more than 5 in 10,000 persons in the European Union.

5. HMPC** (Committee for Herbal Medicinal Products):
- participation in other activities including consultations on the information produced by the committee

□ □□ Interested and my org. has all the necessary resources
□ □□ Interested, but my org. does not have the necessary resources
□ □□ Not interested
Comments:

6. PDCO* (Paediatric Committee):
- participation as member/observer

□ □□ Interested and my org. has all the necessary resources
□ □□ Interested, but my org. does not have the necessary resources
□ □□ Not interested
Comments:

** The HMPC (Committee for Herbal Medicinal Products) has the main task of assisting EU members states and European institutions with scientific opinions related to herbal medicinal products
* The Paediatric Committee is responsible for providing opinions on the development of medicines for use in children, in accordance with the legislation.

1. Activities related to the information on medicines:
- participation as expert for the review

The AGENCY is willing to provide information in a way that fulfils the patients'/general public's expectation. For this reason it has set up procedures to involve Patients' and Consumers' Organisations in the review of information on medicines, addressed to the public including:
Review of EPAR summaries
Review of Package Leaflets
Review on Q&As on safety
Review on Q&As on withdrawal or refusal of marketing authorisation applications

☐ ☐☐ Interested and my org. has all the necessary resources
☐ ☐☐ Interested, but my org. does not have the necessary resources
☐ ☐☐ Not interested
Comments:

1. Please indicate, according to your organisation prferences and resources available, which activities you would like to be included first

Rate
1st activity
2nd activity
3rd activity

Thank you for completing this survey. The outcome of this survey will be discussed and it will be taken into account for future planning.

Annex 4

London, 13 February 2009
Doc. Ref.: AGENCY/483439/2008 *rev. 1*

RULES OF INVOLVEMENT OF MEMBERS OF PATIENTS' / CONSUMERS' AND HEALTHCARE PROFESSIONALS' ORGANISATIONS IN COMMITTEES RELATED ACTIVITIES*

DISCUSSION WITH THE AGENCY/CHMP WORKING GROUP WITH PATIENTS' AND CONSUMERS' ORGANISATIONS	December 2005
ADOPTION BY CHMP, COMP AND HMPC	January 2006
RELEASE FOR CONSULTATION	26 January 2006
DEADLINE FOR COMMENTS	30 March 2006
ADOPTION BY CHMP	31 May 2006
ADOPTION BY COMP AND HMPC	12 July 2006
REVISION DISCUSSED WITH THE AGENCY/CHMP WORKING GROUP WITH HEALTHCARE PROFESSIONALS' ORGANISATIONS	30 October 2008
REVISION DISCUSSED WITH THE AGENCY SCIENTIFIC COMMITTEES' WORKING PARTY WITH PATIENTS' AND CONSUMERS' ORGANISATIONS	27 November 2008
REVISED DOCUMENT ADOPTED BY CHMP, COMP, HMPC, PDCO, CAT	13 February 2009

***This document is a revision of 'Rules of involvement of members of Patients' and/or Consumers' Organisations in Committees related activities' (AGENCY/483439/2008) to make these rules also applicable to Healthcare Professionals' Organisations.**

Rules of involvement of members of Patients'/Consumers' and Healthcare Professionals' Organisations in Committees related activities

1. Background and legal basis

The new Community legislation gives additional responsibilities to the Agency, its Management Board and its Scientific Committees to develop contacts with its various stakeholders. The objective is to provide better information and facilitate communication and dialogue on matters of common interest relating to medicinal products.

Article 78 (1) and 78 (2) in Title IV of Regulation (EC) No 726/2004 provide as follows:

The Management Board shall, in agreement with the Commission, develop appropriate contacts between the Agency and the representatives of the industry, consumers and patients and the health professions. These contacts may include the participation of observers in certain aspects of the Agency's work, under conditions determined beforehand by the Management Board, in agreement with the Commission.

The committees referred to in Article 56(1) and any working parties and scientific advisory groups established in accordance with that Article, shall in general matters establish contacts, on an advisory basis, with parties concerned with the use of medicinal products, in particular patient organisations and health-care professionals' associations. Rapporteurs appointed by these committees may, on an advisory basis, establish contacts with representatives of patient organisations and health-care professionals' associations relevant to the indication of the medicinal product concerned.

The objective of this procedure is to define different types of consultation, on an advisory basis, of specific Patients'/Consumers' and Healthcare Professionals' Organisation(s) on product, disease or treatment area specific issues.

- by the Scientific Committees (CXMP[1]);
- by the Working Parties or the Scientific Advisory Groups;
- by the Rapporteurs.

2. Conditions for the consultation

General conditions

The consultation will follow the Rules of Procedure of the CXMP/Working Party/Scientific Advisory Group.

The need for a given consultation should be agreed by the CXMP and should be channelled through the AGENCY secretariat for organisation.

The CXMP should decide beforehand whether the Committee wishes to consult Patients'/Consumers' and Healthcare Professionals' Organisations by inviting a representative acting on behalf of that organisation, or a member of a given organisation acting as an individual expert.

[1] CXMP refer to CHMP, COMP, HMPC, PDCO and CAT.

In both cases, the scope of the consultation should be clearly defined and questions to be addressed should be adopted by the Committee. This will help the organisations to identify the right member(s) to attend the discussion and facilitate the preparation of such consultation.

The AGENCY will send the request to the contact person of the organisation for nomination of either member(s) who will represent the organisation or member(s) who will act as individual expert(s). As far as possible the request should be sent well in advance of the meeting to allow the organisation to make the necessary arrangements.

In order to make sure that the AGENCY establishes contacts only with the appropriate organisations, and that it gets contributions which are representative of the members of the organisation at European level,, the consulted organisation(s) will have to fulfil certain criteria defined by the Agency and its Committees[2]. In exceptional cases, if justified, the CXMP may decide to consult an organisation not fulfilling the criteria. However such an organisation should still be fully transparent with regard to its activities and funding.

The member(s) nominated by the organisation to participate either as representative(s) of the organisation or as expert(s), will have to adhere to the AGENCY Code of Conduct and to the provisions defined in the AGENCY policy on the Handling of Conflict of Interests. Additional specific conditions will apply, depending on whether the organisation's member(s) are invited as representative(s) of their organisation or acting as individual expert(s).

Specific conditions

Patients/consumers or healthcare professionals as representatives of their organisation

Patients/consumers or healthcare professionals representing their organisation will have the responsibility to liaise with the organisation as necessary in order to deliver the position of the organisation on the questions to be addressed.

In case of involvement in a product related activity, the applicant/sponsor/MAH will be informed in advance about the Stakeholder Organisation consultation and of any list of questions being put to them. In this case, confidential data could be brought to the knowledge of the concerned organisation. The agreement of the applicant/sponsor/MAH should be sought prior to disclosure of these confidential data.

In case the applicant/sponsor/MAH does not agree to a consultation with representative(s) of a Patients'/Consumers' or Healthcare Professionals' Organisation, this will be made public at the end of the evaluation procedure as part of the usual publication on outcomes (e.g. EPAR, publication on withdrawal or negative opinion). Where the applicant/sponsor/MAH submits reasons for this refusal, these will also be published.

Patients/consumers or healthcare professionals as experts

Patients/consumers or healthcare professionals may be invited as an individual expert, and therefore not representing his/her organisation. He/she will have to adhere to the same rules as all other experts participating in AGENCY activities, especially with regard to confidentiality undertaking and will have to adhere to the AGENCY Code of Conduct.

[2] For patients' and consumers' organisations: "Criteria to be fulfilled by Patients' and Consumers' Organisations involved in AGENCY Activities" (AGENCY/14610/04/Final).
For healthcare professionals' organisations: "Criteria to be fulfilled by Healthcare Professionals' Organisations involved in AGENCY Activities"(AGENCY//)

No documentation will be sent prior having received the signed form(s) (i.e. declaration of interest and confidentiality undertaking).

The balance between confidentiality and the need for sharing the information within the organisation concerned is recognised. Therefore if for technical reasons the member needs the opinion of colleagues from his/her organisation in order to prepare for the discussion, he/she should inform the AGENCY Secretariat and send the names of the additional members with whom the information would be shared. They would have to respect the same rules before they could have access to any information, in particular all the members would have to sign the confidentiality undertaking.

In case of involvement in product related activity, the applicant/sponsor/MAH will be informed of the names of the person(s) in accordance with the same rules as for any other expert consulted. In case the person acting as expert is approached by the applicant/sponsor/MAH, he/she should immediately contact the AGENCY.

In accordance to article 62(2) of Regulation (EC) No 726/2004, when involved as expert, the member of the patients'/consumers' and healthcare professionals' organisation will be entered in the AGENCY EU experts' database.

3. Consultation

Within the patients'/consumers' and healthcare professionals' organisations, most members are subject to constraints in terms of time, budget and availability for travelling to the AGENCY. Therefore the relevance of their involvement in such CXMP activities will be carefully assessed before any decision on their involvement can be taken. In addition, alternative tools for interaction will be envisaged as far as possible including written procedure, video link or teleconference. However, when the presence as experts or as representatives of patients'/consumers' and healthcare professionals' organisations is deemed necessary, the meeting will take place at the AGENCY offices in London.

As far as possible, the AGENCY will send the relevant documentation well in advance of the meeting to allow sufficient time for preparation. The timeframe will be adapted to the procedure involved.

In accordance with the Rules of Procedures, the Committee/ Working Party/Scientific Advisory Group shall neither conduct any deliberations nor reach any formal opinions or decisions in the presence of representatives from any organisation. This does not apply to the situation when such representatives are involved as individual experts.

Whether the patient(s)/consumer(s) or healthcare professional(s) were invited as representative of their association or as expert, the relevant section of the minutes will be circulated to them for comments, excluding the section on the deliberations or any formal opinions or decisions reached by Committee/ Working Party/Scientific Advisory Group. The minutes or the outcome of the discussion will be forwarded to the Committee
In case of consultations taking place directly with a Rapporteur or a Working Party/Scientific Advisory Group, a report on the outcome of such contacts should be provided to the Committee.

If the organisations' representatives wish to keep some information confidential (such as private information which cannot be anonymised) from the company concerned, they should state it clearly and this should be recorded in the minutes of the meeting.

If the organisations' representative(s) were consulted on a centralised procedure, this consultation will be reflected in the public assessment report (e.g. EPAR, withdrawal assessment report).

Expenses related to travel and accommodation regarding the meeting (i.e. travel arrangement, hotels, daily allowance) will be covered by the AGENCY in accordance to the AGENCY rules for reimbursement. In case of patients and consumers, specific implementation rules may apply.

Annex 5

Current experience and practice as regards compensation for the work performed by patients and consumers at European and national level

The experience of other European Agencies: the example of EFSA

The basis for reimbursement for delegates and experts (without specific distinction for consumers) who participate in their activities through participation in meetings is similar to the AGENCY, and covers travel and hotel costs.

However, although no specific formal financial compensation is provided, daily allowance amounts to a total of 300 euro for each day of participation to every delegate or expert. This amount is provided despite being the estimated cost of living in Parma lower than in London.

A particular case in EFSA refers to the "Stakeholder Consultative Platform", a platform which assists EFSA with the development of its overall relations and policy with regards to stakeholders' involvement. In this case, the costs of the participation in each meeting are borne by each individual organisation attending. Exceptionally, EFSA may contribute to financing the costs of those organisations that could not otherwise afford to participate. Requests for financial support are assessed on a case-by-case basis and are properly documented.

The experience at national level

Patient/consumer participation into regulatory activities and its possible compensation has been explored in different national agencies:

- **France** - Patients' organisations are gradually represented in a number of Afssaps' committees and working groups. They are subject to the same rules of reimbursement as any other external experts and delegates. No financial compensation is foreseen neither for the work they perform nor in case they leave their daily work.

- **United Kingdom** - Experience exists in involving patients in the work of MHRA. Reimbursement covers travel and hotel expenses and provides a daily allowance which rates differently depending on the activity to be performed.

  Additionally and upon request, they cover for any expense which may be derived from their attendance to the meeting (i.e. locum general practitioner, nanny (for childcare costs etc). This approach applies to all experts and delegates with no distinction for patients and consumers.

- **Sweden** - MPA has experience involving patients in their activities, and they are reimbursed for travel and accommodation as any external expert. No other specific provision applies.

- **The Netherlands** - Increasing experience involving patients. They are reimbursed for travel and accommodation as any external expert. No other specific provision applies.

- **Italy** - Not much experience with patients as experts so far. No specific provision applies for compensation.

- **Denmark** - Not much experience with patients as experts so far. No specific provision applies for compensation.

- **Spain** - Not much experience with patients as experts so far. No specific provision applies for compensation.