EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

16 September 2010
EMA/MB/579729/2010

Third report on the progress of the interaction with Patients' and Consumers' Organisations during 2009

Management Board meeting 7 October 2010

Background note

The present report describes the continuous progress of the interaction between the European Medicines Agency and Patients' and Consumers' Organisations (PCOs) during 2009. It also includes the status of implementation of the actions and recommendations that were identified in previous reports.

Matters for consideration

During 2009 there has been a continued increase in the number of Patients/Consumers' Organisations representatives who have been involved in EMA activities (**77** in 2007, to **165** in 2008, to **213** in 2009). This can be attributed to an increased involvement at all levels of the Agency's work (e.g. more members in Scientific Committees, more participation of patient experts in Scientific Advisory Group (SAG) meetings, involvement in Working Parties (WPs) and other EMA events and workshops, as well as an increased involvement in the preparation of documents).

Following an analysis of the experience in 2009 and having considered the proposals for action endorsed by the Management Board in 2009 as part of the "Reflection Paper", the next steps in the interaction are as follows:

- The "Framework of Interaction" between the EMA and PCOs will be extensively revised during 2010/11. A new set of performance indicators will be prepared to measure the impact of the activities proposed in the "Reflection Paper" (including the provision of financial support for their participation).
- PCOs representatives will continue to be involved in the preparation of information oriented to patients and the general public. In particular the Agency will look at their input in the preparation of additional EMA information (i.e. emerging safety information) and the best way to ensure timely and effective dissemination of this information to the concerned European Organisations.
- The Agency will provide further training in several areas (e.g. regulatory procedures, clinical trials, innovative medicines) which are considered of great interest by Patients'/Consumers' Organisations.

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- The Agency will also explore how patients and consumers can be involved in the benefit/risk assessment of medicinal products, defining the criteria for their involvement. The role of patients and consumers in the different Scientific Committees of the Agency will also be defined.
- In 2011 the Management Board will be presented with the report on the progress achieved in 2010.

The present report was presented to the Patients' and Consumers' Organisations Working Party (PCWP) during its meeting on 16th June 2010 and to the EMA Management Board on 7th October 2010.

EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

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Third report on the progress of the interaction with Patients' and Consumers' Organisations during 2009

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1. Executive summary

Introduction

In 2005, at the time of endorsing a specific framework in the field of the interaction between the European Medicines Agency (EMA, or 'the Agency') and patients' and consumers' organisations (PCOs) (EMEA/354515/2005-Final), the EMA Management Board (MB) requested that an annual report on the progress of the interaction should be presented to the Board in order to monitor the progress of the activities defined in the above-mentioned framework.

The present report describes the continuous progress of the interaction during 2009, including the status of implementation of the actions and recommendations that were identified in previous reports. Also included is the progress of the work done by the *EMA Human Scientific Committees' Working Party with Patients' and Consumers' Organisations* (PCWP), as well as the activities related to the provision of information to patients and the general public. 2009 has also been dedicated to the development of a reflection paper on the further involvement of PCOs in the activities of the Agency, which has been published in December 2009.

The present report was presented to the PCWP during its meeting on 16th June 2010 and to the EMA Management Board on 7th October 2010.

Progress on the interaction with Patients' and Consumers' Organisations during 2009

2009 has seen a continued increase in the number of patients/consumers' organisations representatives who have been involved in EMA activities (from **77** in 2007, to **165** in 2008, to **213** in 2009). This can be attributed to an increased involvement at all levels of the Agency work (e.g. more members in scientific committees, more participation of patient experts in Scientific Advisory Group (SAG) meetings, involvement in working parties (WPs) and other EMA events and workshops, as well as an increased involvement in the preparation of documents).

The conclusions from previous reports have provided evidence that all actions identified in the 'Framework of interaction' have been implemented and interaction has been established between patients'/consumers' organisations and the EMA Management Board, the EMA Scientific Committees, Working Parties, Working Groups and Scientific Advisory Groups. However, although the groundwork is established, there remains the need to further improve on the procedural aspects of this interaction in certain areas of the Agency's work. In this regard, within the scope of the first two reports endorsed by the Management Board, the EMA has been working during 2009 on the preparation of a "Reflection paper on the further involvement of patients and consumers in EMA activities", which proposes specific actions for a more formal and systematic interaction. The reflection paper has been endorsed by the EMA Management Board in December 2009.

With regard to the provision of information on medicinal products, the Agency has continued implementing measures aimed at improving the quality of product-related information adapted and oriented to patients and the general public. The scope of the review procedure, in place since 2007, has been extended during 2009, so that now the Agency involves PCOs representatives in the preparation of a wider range of documents (i.e. EPAR summaries, Package Leaflets, Q&A documents).

2009 also saw a pilot exercise where patients participated as observers in 3 consecutive meetings of the PhVWP. The analyses of the experience resulted in a report and a formal proposal for the

participation of patients as regular observers in the PhVWP. This proposal is being implemented in 2010.

The results of the 2008 performance indicators questionnaire have been used to identify areas for improvement and to propose specific actions, which have been considered during the finalisation of the aforementioned reflection paper. The implementation of these actions started in 2009 and will continue during 2010, as some of the actions have been incorporated in the PCWP Work Programme for 2010.

One of the actions proposed in 2008 concerned the enlargement of the PCWP. Following this recommendation, and considering the need to involve more patients'/consumers' organisations able to cover additional therapeutic areas, the process for the enlargement of the PCWP has been initiated in 2009 and concluded in early 2010.

Next steps

- Following the endorsement by the Management Board of the proposals for action in the "Reflection Paper", implementation started in January 2010.
- The "Framework of interaction" between the EMA and PCOs will be extensively revised during 2010. A new set of performance indicators will be elaborated to measure the impact of the activities proposed in the reflection paper (including the provision of financial support to their participation).
- PCOs representatives will continue to be involved in the preparation of information oriented to patients and the general public.
- The Agency will explore the best way to involve patients/consumers in the preparation of additional EMA information addressed to patients and the general public (i.e. emerging safety information) and the best way to ensure timely and effective dissemination of this information to the concerned European organisations.
- The Agency will provide further training in several areas (e.g. regulatory procedures, clinical trials, innovative medicines) which are considered of great interest by patients'/consumers' organisations.
- The Agency will also explore how patients and consumers can be involved in the benefit/risk assessment of medicinal products, defining the criteria for their involvement. The role of patients and consumers in the different scientific committees of the Agency will also be defined.
- The Management Board will be presented in 2011 with the report on the progress achieved in 2010.

2. Overview of patients' and consumers' organisations involvement in the activities of the European Medicines Agency during 2009

This report describes the different kinds of EMA activities in which PCOs representatives participated in 2009, and the different contributions made. It includes comparative analyses with the level of involvement during 2007 and 2008. It also describes how the actions identified in previous reports have been implemented, and identifies the next steps in the interaction for the coming years.

Based on previous progress reports, a '*Reflection paper on the further involvement of patients and consumers in EMA activities*' was prepared. It was endorsed by the Agency Management Board in December 2009. It focuses on the best way to further develop the interaction between the Agency and patients'/consumers' organisations, taking into account the experience gained in the previous years and the interests and capabilities of the eligible PCOs. This paper contains two specific proposals: the first concerns the need to revise the current framework of interaction, and the second is aimed at providing financial support in specific cases to experts and delegates from patients'/consumers' organisations.

Since 2008, the Agency has incorporated more patients as formal members in its Scientific Committees (COMP, PDCO, CAT). In 2009, the Agency started to explore how to involve patients and consumers in the activities of the CHMP and its working parties. In this context, patients'/consumers' representatives participated, during a three-month pilot phase, in three consecutive meetings of the CHMP Pharmacovigilance Working Party (PhVWP). This pilot phase resulted in a proposal, endorsed by the Management Board, for the permanent inclusion of patients'/consumers' representatives in the PhVWP (see pages 8-9 for more details).

The Agency has also consulted patients on policy issues, product-related matters and dissemination and preparation of information, etc. These activities have usually been supported through the work of the PCWP, but a wider network of organisations eligible to work with the European Medicines Agency has also been involved in many Agency activities (see Table 1 on page 13 for the full list of activities).

Over the years, the Agency has been building a 'Network of experts' from patients' and consumers' organisations. This network, which now comprises over 100 experts, includes all experts routinely involved in the review of product information, as well as experts involved with the Agency on an 'ad hoc' basis (workshops and conferences, SAG meetings etc.). This network is an extremely valuable resource for the Agency, and has been constantly expanding due to the increasing number of organisations eligible to participate in EMA activities. On the basis of their therapeutic area of interest, each eligible organisation (evaluated against a defined set of criteria) can be contacted by the Agency and asked to contribute, according to its resources, to different activities/initiatives. The added value of the contribution of 'lay experts' to the activities of the Agency has been acknowledged on several occasions, and the Agency aims to strengthen and further develop this interaction. An up-to-date list of eligible organisations is published on the Agency website.

2.1. EMA Management Board (MB)

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

The first mandate of three years was completed on 27 September 2008 by Mary Baker from the European Federation of Neurological Associations (EFNA) and Jean Georges from Alzheimer Europe (AE). For the current term of three years the PCOs representatives are Mary Baker and

Mike O'Donovan from the European Patients Forum, and they were officially nominated during the MB meeting on 5 March 2009. The representatives of patients' organisations 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.

A member from the Management Board has been attending the PCWP meetings during 2009 as an observer in order to maintain a link between the two groups.

2.2. EMA Scientific Committees

During 2009 patients and consumers have been formal members of three EMA Scientific Committees (the COMP, the CAT and the PDCO). Additionally, all five Human Committees have consulted PCOs representatives on specific issues when needed.

2.2.1. Committee for Orphan Medicinal Products (COMP)

The COMP (as per Article 4 (3) of Regulation (EC) Nº 141/2000) includes in its membership "three members nominated by the Commission to represent patients' organisations". They have been nominated by the European Commission on 1 July 2009 for a term of three years, which shall be renewable. Their tasks during 2009 have included, among others:

- Participation in the assessment of applications for Orphan Drug Designations and acting as coordinators for some of the applications;
- Providing advice on the identification of external experts when needed for the assessment of the Orphan Drug Assessments;
- Collaboration in the preparation of public summaries of opinion (PSOs) for orphan designations.

2.2.2. Paediatric Committee (PDCO)

The PDCO (as per Article 4 (1.d) of Regulation (EC) Nº 1901/2006) shall include in its membership "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". They have been nominated by the Commission on 31 July 2008 for a renewable term of three years; their tasks during 2009 have included among others:

- Participation in the peer reviews of ongoing Paediatric Investigation Plan (PIP) applications, including presentation of their conclusions to the Committee in relation to the assessment of the PIP;
- Participation as PDCO representatives in other Agency activities.

2.2.3. Committee for Advanced Therapies (CAT)

The composition of the Committee for Advanced Therapies comprises "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" (as foreseen in Article 21 (1.d) of Regulation (EC) Nº 1394/2007). They have been nominated by the European Commission at the end of 2008 for a renewable period of three years. The first meeting of the CAT was held on 15-16 January 2009.

Patients' representatives, as members of the CAT, have been involved in several committee activities as per the regulation of advanced therapy medicinal products.

2.2.4. Committee on Herbal Medicinal Products (HMPC)

There is currently no legal basis in the European legislation for patients' membership in this Committee, and any interaction is established through provisions 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 the link between the two groups.

The HMPC has consulted the PCWP on various issues, and several discussions on how best to improve the way the Agency communicates information on herbal medicines to the general public have been held. In this context, a joint meeting of the HMPC and representatives of patients'/consumers' organisations took place on 13 March 2009. It emerged from this meeting that the information on herbal medicines that the Agency produces is clearly of interest to patients and the general public, and that PCO representatives with a specific interest/expertise in herbal medicines could contribute to the preparation of this type of information.

2.2.5. Committee for Medicinal Products for Human Use (CHMP)

There is currently no legal basis for patients' membership in this Committee. Interaction with the CHMP and its Working Parties and Scientific Advisory Groups (SAGs) is based on Article 78, indent (18) of Regulation (EC) Nº 726/2004. Participation of PCOs representatives has occurred in the following ways:

- Ad-hoc meetings on issues related to products under evaluation

In 2009 patients' representatives participated in two meetings in which the EMA updated patients and victims of thalidomide with the latest status and information for thalidomide, as agreed during the previous "Meeting of victims' and patients' organisations on Thalidomide", held in 2007. The EMA will continue to update patients' organisations on this issue whenever necessary.

- Written consultations of eligible patients' and consumers' organisations

The CHMP requested the views of patients and consumers on medicinal products under evaluation. This occurred three times in 2009; for the medicinal products Onsenal, Prezista and Tysabri. In each case, selected patients' organisations, fulfilling the Agency's criteria for interaction, were asked to answer in writing a list of questions adopted by the Committee, and in some cases were subsequently invited to participate in the CHMP discussion on the issues. These initiatives gathered the experience and views of the organisations on certain aspects of the current use of medicines, and this information has been taken into account by the CHMP on the opinions subsequently adopted and has been reflected in the CHMP assessment report.

- Participation in SAG meetings

SAGs are groups of experts convened by the CHMP to provide advice during the evaluation of a specific product or treatment. During 2009, three patients were invited to participate and contribute as experts in three different Scientific Advisory Group (SAG) meetings to give their views on specific questions (on bisphosphonates, on gadolinium-containing products and on natalizumab and the risk of PML).

The CHMP reviewed the work carried out by the Scientific Advisory Groups during 2008/09, and the subsequent analysis has been used in 2010 to propose to involve patients regularly in SAGs meetings during a pilot phase of 1 year.

- Participation in the Scientific Advice Working Party (SAWP)

Patients participated as experts in 3 procedures for scientific advice during 2009.

- Participation to the Pharmacovigilance Working Party (PhVWP) activities

Following the preparatory work done in 2008, the Agency Management Board endorsed in March 2009 a proposal for the involvement of patients'/consumers' representatives in three consecutive meetings of the CHMP Pharmacovigilance Working Party (PhVWP). This 'pilot phase' exercise covered the PhVWP meetings in April, May and June 2009, and two patients'/consumers' representative participated as observers to the meetings. The patients'/consumers' who attended the PhVWP, together with the collaboration from members of the PhVWP, the PCWP and the Agency secretariat, prepared a report analysing the outcome of the pilot exercise. This report included a formal proposal for involvement and participation of patients'/consumers' representatives in the meetings of the PhVWP, which has been endorsed by the Agency Management Board and by the Heads of Medicines Agencies (HMA). The final report has been published in December 2009. In the report it was agreed that, after a call for expression of interest and a selection procedure, the Agency will nominate one patients'/consumers' representative to join the PhVWP as an observer; a second representative will stand as alternate. Full implementation started on 1st May 2010.

The Pharmacovigilance Working Party (PhVWP) has sought the views of the patients and consumers on several proposed changes to the wording of the Package Leaflets and consulted them as experts on three occasions (for products containing orlistat, apomorphine, and vigabatrin).

2.3. EMA Working Party with Patients' and Consumers' Organisations (PCWP)

The PCWP has continued to play an essential role in the progress of the interaction between the European Medicines Agency and patients'/consumers' organisations. The composition of the PCWP during 2009 was as follows:

- 10 members and 8 alternates representing PCOs;
- 4 members from the EMA Scientific Committees (CHMP, COMP, PDCO, HMPC);
- Observers from the CMD-h, the HCP WG and the Management Board.

The activities of the PCWP during 2009 included four plenary meetings, including one meeting with all organisations which fulfil EMA eligibility criteria, and one joint meeting with the Healthcare Professionals' Working Group (HCP WG). Also, PCWP and HCP WG members participated in a dedicated meeting with the Head of Pharmaceuticals unit (DG Enterprise and Industry, European Commission) to discuss the new EU pharmaceutical legislation (see page 11). In addition, a one-day training session for all experts involved in the review of product-related information was organised. The PCWP was also consulted by other groups (PhVWP and CMD (h)) on the PL wording of certain medicines (e.g. orlistat, apomorphine).

Through the PCWP, patients and consumers representatives have been involved in other projects, as in the case of the Innovative Medicines Initiative (IMI), the European Network of Centres for Pharmacoepidemiology and Pharmacovigilance (ENCePP), the Pharmacoepidemiological Research on Outcomes of Therapeutics (PROTECT).

2.4. Activities related to Clinical Trials

2.4.1. EMA 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" (EMEA/228067/2008), a Working Group on Third Country

Clinical Trials has been established within the Agency. The Working Group includes in its composition representatives from the PCWP, who have been integrated as any other members in the different topics within the Action Plan 2008-2011. Patients'/consumers' representatives are members of four different sub-groups, and each group has worked on drafting recommendations on several areas related to the regulation of clinical trials conducted in third countries. The outcome of the work performed by this working group will be illustrated in a reflection paper that was released for public consultation in 2Q 2010; also, a workshop on this topic was held in September 2010.

2.4.2. Development of EudraCT

In addition to the involvement in the above-mentioned Working Party, patients'/consumers' representatives have also participated to the 'EudraCT information day' on 30 April 2009, during which an update was given on the development of the new public interface of EudraCT; the European database containing information on clinical trials currently ongoing in the EU, which is expected to be available to the public in 2011.

2.5. Activities related to the provision of information to patients and the general public

The European Medicines Agency is responsible for providing information about medicines authorised via the centralised procedure, which includes information directed to the patients and the general public. During the preparation of this information, the Agency interacts with patients' and consumers' organisations to ensure that it is adequately formulated and comprehensible to the target audience. The purpose of this consultation is to ensure that the information is clear and understandable, and that it fulfils the needs of patients in terms of information content.

Patients and consumers have been regularly involved in several aspects of the provision of information on medicinal products. The activities undertaken include feedback from patients on the readability of the information contained in package leaflets (PLs), EPAR summaries, public statements and similar materials intended for the public.

The procedure for the review of EPAR summaries and PLs at the time of renewal of the marketing authorisation was implemented as of May 2007 (EMA/279083/2006 Rev 1), following the identification of a list of experts and the organisation of a training session on the review procedure.

The analysis of the experience acquired so far demonstrates that the majority of comments received are of high quality and useful, and that in general patients' and consumers' contribution increases the quality of the documents within the scope of this procedure.

- Review of EPAR summaries

During 2009 PCOs representatives reviewed a total of 36 new EPAR summaries.

- Review of Package Leaflets

During 2009, PCOs representatives reviewed a total of 33 Package Leaflets (PLs). This includes PLs at the time of renewal and also at the time of the initial evaluation of the marketing authorisation application.

- Training on the 'Review of European Medicines Agency documents addressed to the general public by patients and consumers'

The annual training session on the review of EMA documents addressed to the patients and the general public (namely, package leaflets and EPAR summaries) has been held at the Agency on 7 December

2009. Patients and consumers experts were trained on the practical and theoretical aspects of the review, and they had the chance to review sample documents and to get feedback on their comments from the medical writers and the QRD members responsible for the preparation and quality check of said documents. The experts, representing all the organisations eligible to interact with the Agency, were provided with a CD ROM and a training manual containing all the information needed to perform the review of the documents. Upon request from the PCOs representatives, the EMA will explore in 2010 the possibility to expand this training and to make it available to more patients and consumers.

- Involvement in the preparation of EMA safety communications

The Agency communicates emerging safety issues to the general public, usually in the form of Q&A documents and press releases. Patients have been involved in the preparation and dissemination of EMA communications on selected issues (e.g. for product shortages related to orphan drugs). The feedback received on this interaction is very positive, as patient contribution results in an added value to the outcome. PCOs involvement in this area will be further developed in 2010 following the revision of the 'Framework of interaction' between the Agency and patients'/consumers' organisations. More specifically, a system will be set up, which will guarantee that product safety announcements together with other critical issues communicated by the Agency reach the concerned stakeholders in a timely manner.

- Involvement in the preparation of other documents addressed to the general public

Patients and consumers in 2009 have participated in the preparation of other documents directed to patients and the general public, such as the "Questions and answers on generics" and the "Questions and answers on the regulation of advanced therapy medicinal products".

- Provision of additional information in relation to the Agency's activities

PCOs representatives were also involved during 2009 in the development of the Agency new website, carrying out user-testing on the prototype website. They also contributed to the development of the Agency new corporate identity through a survey which was distributed to all stakeholders.

2.6. Other activities

- Meeting with the Head of Pharmaceuticals unit (DG Enterprise and Industry, European Commission) to discuss the new EU pharmaceutical legislation

On 29 September 2009, patients' and consumers' representatives, together with representatives of the healthcare professionals, took part in a meeting with the Head of Pharmaceuticals unit of the European Commission, to discuss the new EC legislative proposals regarding the pharmaceutical sector. The proposals on Counterfeit medicines, on Pharmacovigilance and on Information to patients (which constitutes the so-called 'pharma package') were presented and discussed, and the feedback received from patients, consumers and healthcare professionals has been considered of great value by the EC representative.

- Involvement in the Agency transparency projects

Various Agency projects in relation to transparency have been subject to specific consultation with patients. Examples of these consultations include the new EMA Transparency Policy which also included participation in two workshops organised by the Agency on this topic.

These kinds of initiatives have been much appreciated and their value has been recognised by both the Agency and the stakeholders, as they provide useful input on basic principles of complex projects at an early stage of development.

- Involvement in ENCePP

Patients'/consumers' representatives participated in the meetings of the European Network of Centres for Pharmacoepidemiology and Pharmacovigilance (ENCePP). The aims of this initiative for 2009 were to put in place an operational network system that would allow the conduct of "ENCePP studies"; to set up a Code of Conduct setting out rules for transparency and scientific independence in pharmacovigilance and pharmacoepidemiology research; and to agree on the mandate which will enable the appointment of the ENCePP Steering Group. Regarding this last point, one patients'/consumers' representative was nominated as a member of the steering group at the beginning of 2010.

- EMA Conferences and Workshops

PCOs representatives have also participated in an increasing number of events organised by the Agency in comparison with 2007 and 2008. See Table 1 on the next page for the full list of activities.

Table 1. Activities involving patients/consumers at the European Medicines Agency during 2009

Management Board/Scientific Committees
MB (members)
COMP (members and observers)
PDCO (members)
CAT (members)
CHMP consultation on medicinal products under evaluation
Working Parties/Working Groups
PCWP (members and observers)
PhVWP – observers during a three-month pilot phase
SAWP – participation as experts in the review of SA requests
HCP WG (observers)
Working Group on Clinical Trials in Third Countries (members)
PhVWP experts consultations on medicinal products
PhVWP consultation on PL wording of medicinal products
Ad Hoc/SAG meetings
SAGs – participation as experts
Internal meetings with Thalidomide UK on update on the use of thalidomide products
Internal meeting with ENFA on fibromyalgia
Product information related activities
Review of Package Leaflets (new and renewal MA applications)
Review of EPAR summaries
Training on the review of documents addressed to patients and the general public
Review of Q&A documents on generics and on advanced therapies
Review of Q&A documents on safety communications
Workshops
EMA/TREAT-NMD Workshop on Duchenne Muscular Dystrophy
Workshops on EMA Transparency Policy
Joint PCWP/HMPC workshop on development of HMPC ARSP
Experts meeting on Biomarkers for Alzheimer
Other meetings
European Network of Centres for Pharmacoepidemiology and Pharmacovigilance (ENCePP)
Pharmacoepidemiological Research on Outcomes of Therapeutics – Innovative Medicines Initiative (PROTECT – IMI)
EudraCT information day
Meeting with the European Commission on the Pharma-package
Input on other projects
Public consultation on EMA Transparency Policy
User-testing of the EMA prototype website
Evaluation survey of EMA new corporate identity
Interaction with CHMP on medicinal products under evaluation

3. Organisations involved in the European Medicines Agency activities in 2009

All patients' and consumers' organisations are welcome to express an interest to participate in the activities of the European Medicines Agency. Their involvement helps the Agency to conduct its activities with a proper understanding of patients' and consumers' interests in the field of medicines regulation.

Any interested patients' or consumers' organisation must fulfill the 'Criteria to be fulfilled by patients' and consumers' organisations involved in the European Medicines Agency activities'. These criteria are formulated to ensure that the Agency establishes contacts only with appropriate organisations that are genuinely acting in the interests of European patients and consumers.

A list of the organisations eligible to interact with the EMA is published in the dedicated section 'Working with patients and consumers' within the Agency website. Links to the organisations' websites are also provided, together with a short summary of their expertise and main activities.

A negative outcome does not preclude the organisation to reapply at any time, particularly if the issues raised during the evaluation have been addressed. As of December 2009, 58 organisations have applied for evaluation. Of them, 27 have received a positive outcome, 22 have received a negative one and 8 organisations the application was suspended/withdrawn. Finally 1 application was being assessed. As a result of this exercise, a growing number of patients' and consumers' organisations are now able to participate in the Agency activities. This allows the Agency to have direct contact with a suitably wide range of PCOs, and guarantees that their views represent the needs and concerns of patients and consumers across Europe. All the eligible PCOs are not-for-profit organisations, involved at EU level. Some of them are general umbrella organisations; others have a particular focus on a specific patient/consumer-related area (such as rare diseases, HIV/AIDS etc.).

In accordance to the "Rules of involvement of members of patients'/consumers' organisations in Committees' related activities" (EMEA/483439/2008 rev.1), there have been exceptional cases, when the Committees consulted organisations not fulfilling the criteria. However, those organisations were fully transparent with regard to their activities and funding. In one case during 2009, organisations which had not been evaluated against the defined "criteria" participated in a meeting organised by the Agency. They were national organisations specifically related to the topic of the meeting which had been referred by European "eligible" organisations. They are identified and presented in table 3.

During 2009, a total of 41 patients'/consumers' organisations were involved with the Agency (24 PCOs were involved during 2007, 26 during 2008). Table 2 on the following page gives an overview of the patients' and consumers' organisations which so far fulfil the European Medicines Agency criteria after evaluation, and of the organisations that have been involved in different EMA activities during 2009.

Table 2: Patients' and consumers' organisations working with the European Medicines Agency in 2009

	Name of organisation	Fulfilment of EMA eligibility criteria*	Involvement in 2009	Involvement in 2008	Involvement in 2007
1	Alzheimer Europe (AE)	YES	√	√	√
2	European AIDS Treatment Group (EATG)	YES	√	√	√
3	European Cancer Patient Coalition (ECPC)	YES	√	√	√
4	European Federation of Allergy and Airways Diseases Patients' Associations (EFA)	YES	√		
5	European Federation of Neurological Associations (EFNA)	YES	√	√	√
6	European Genetic Alliances' Network (EGAN)	YES	√	√	√
7	European Heart Network (EHN)	YES	√	√	
8	European Multiple Sclerosis Platform (EMSP)	YES	√	√	
9	European Myeloma Platform (EMP)	YES	√	√	√
10	European Older People's Platform (AGE)	YES	√		
11	European Organisation for Rare Diseases (EURORDIS)	YES	√	√	√
12	European Parkinson's Disease Association (EPDA)	YES	√	√	√
13	European Patients' Forum (EPF)	YES	√	√	√
14	European Public Health Alliance (EPHA)	YES	√	√	√
15	Global Alliance for Mental Illness Advocacy Networks (GAMIAN-Europe)	YES	√		
16	Health Action International (HAI)	YES	√	√	√
17	Insulin Dependent Diabetes Trust (IDDT)	YES	√	√	√
18	International Alliance of Patients' Organizations (IAPO)	YES	√	√	√
19	International Diabetes Federation (IDF)	YES	√	√	√
20	International Patient Organisation for Primary Immunodeficiencies (IPOPI)	YES	√	√	√
21	Myeloma Euronet (ME)	YES	√	√	√

22	Rett Syndrome Europe (RSE)	YES	√	√	
23	Thalassaemia International Federation (TIF)	YES	√	√	
24	The European Consumers' Organisation (BEUC)	YES	√	√	√
25	The International Confederation of Childhood Cancer Parents Organisations (ICCCPO)	YES	√	√	
26	Action Duchenne UK	NO	√		
27	Charley's Fund, USA	NO	√		
28	Cure Duchenne, USA	NO	√		
29	Duchenne Ireland	NO	√		
30	Duchenne Parent Project	NO	√		
31	European Network of Fibromyalgia Associations (ENFA)	NO	√		
32	Forschung und Therapie fuer SMA	NO	√		
33	Gaucher Gesellschaft Deutschland E.V. (GGD E.V.)	NO	√		
34	Muscular Dystrophy Association (MDA)	NO	√	√	
35	Muscular Dystrophy Campaign, UK	NO	√		
36	Parent Project Australia	NO	√		
37	Parent Project Onlus Italy	NO	√		
38	Thalidomide UK	NO	√		√
39	TREAT-NMD Neuromuscular Network	NO	√	√	
40	United Parent Projects Muscular Dystrophy (UPPMD)	NO	√	√	
41	Vaincre la Mucoviscidose	NO	√		

4. Overview of patients and consumers involved in EMA activities during 2009

During 2009, 213 patients/consumers have been involved in the activities of the Agency. In some cases the same patient/consumer participated in more than one activity.

The activities have been split into three categories; activities in which patients/consumers are members (e.g. of committees/working parties), activities requiring experts, and activities requiring organisations' representatives.

Table 3: Activities involving patients/consumers at the European Medicines Agency during 2009

Activities requiring membership of committee/working party	Nº of Members / Observers / Alternates
Members of the Management Board	2
Members and observers of the COMP	5
Members and alternates of the PDCO	3+1
Members and alternates of the CAT	2+2
Members and alternates on the PCWP	10+8
Observers in the HCP WG	2
Pilot phase observers at PhVWP	2
Total	**37**

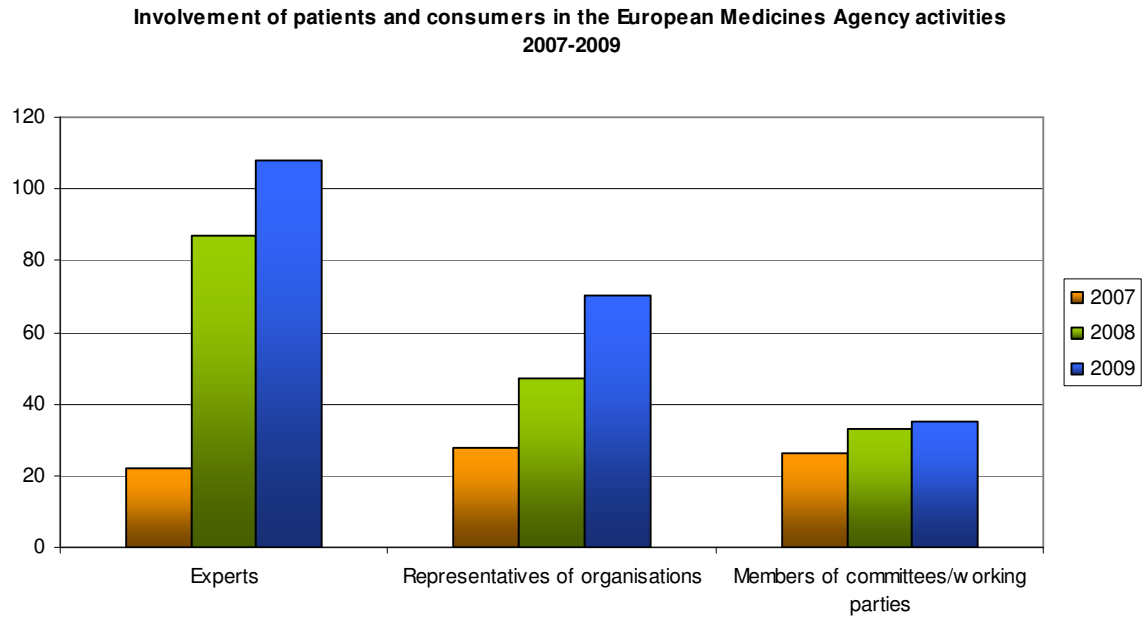
Activities requiring experts	Nº of Experts
Working Group on Clinical Trials in third countries	6
Other experts who attended PCWP meetings	2
Internal meetings with Thalidomide UK on update on use of thalidomide products	2
User-testing for prototype website	3
SAG meetings	3
PhVWP consultations	3
SAWP meetings	3
Q&A preparation (consultation on safety communication)	2
EMA / TREAT NMD workshop on Duchenne Muscular Dystrophy	15
Review of EPAR summaries	36
Review of Package Leaflets	33
Total	**108**

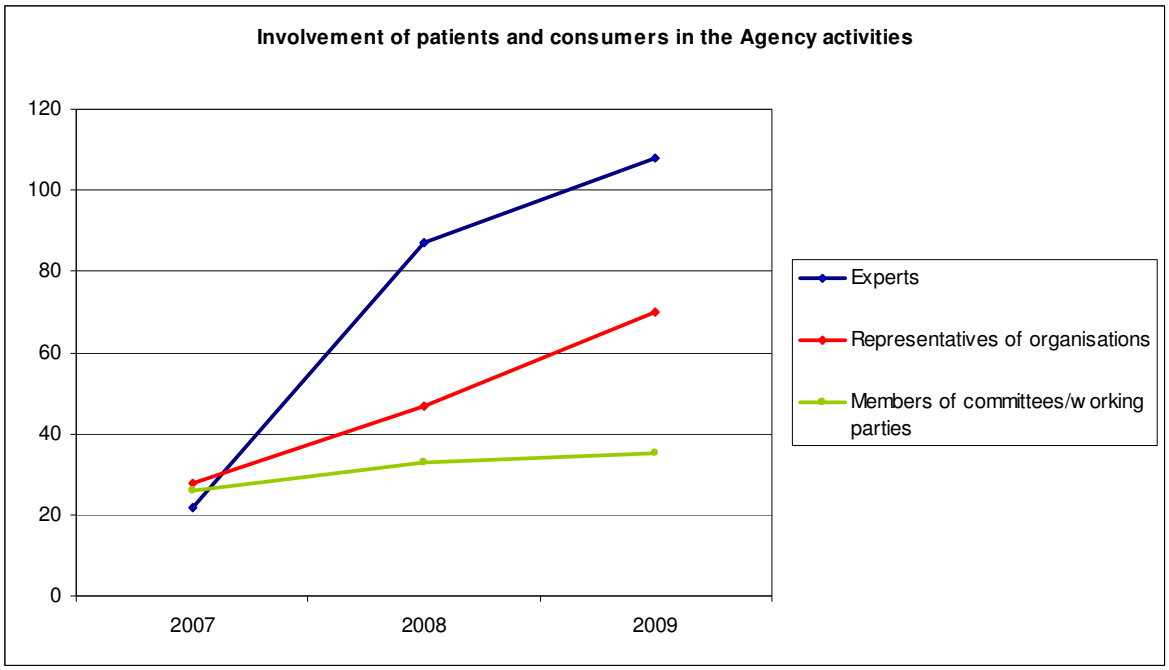
Activities requiring organisations' representatives	Nº of Representatives
Meeting with the EC on the Pharma-package	12
PhVWP consultations	16
Input on EMA corporate identity	10
Workshop on information on herbal medicines	6
Input in the preparation of the Q&A on advanced therapies	1
ENCePP	1
PROTECT	1
First Workshop on Transparency policy	4

Second Workshop on Transparency policy	6
EMA/ENFA meeting on fibromyalgia	3
EudraCT Information day	3
CHMP consultation on products under evaluation	3
Interaction with CHMP on products under evaluation	2
Total	**68**
Total number	**213**

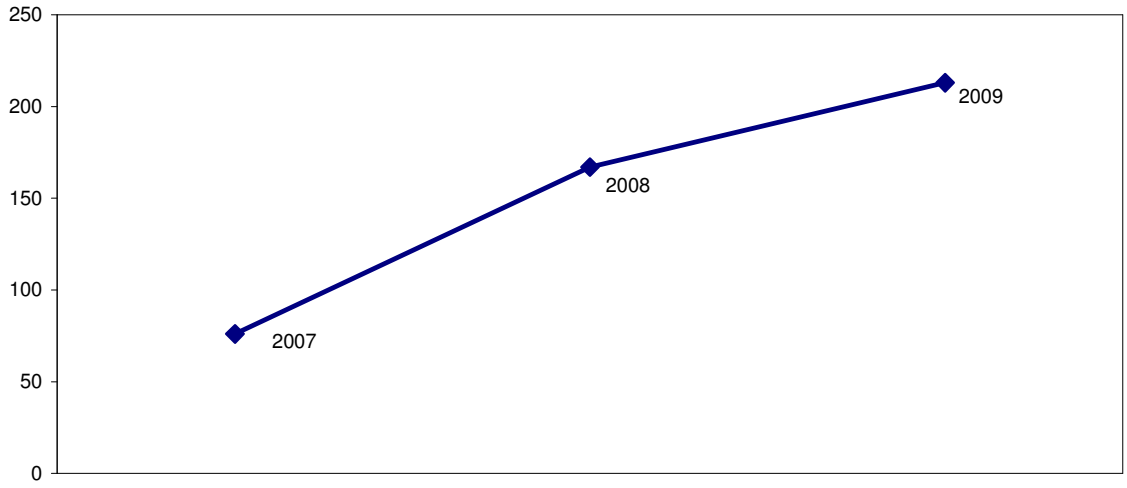
4.1. Involvement of patients/consumers in EMA activities: comparative analysis of data from previous years.

In the graphs below, the number of patients/consumers involved in the activities of the Agency as experts, as PCOs representatives and as committees'/working parties' members during 2009 is compared with those for 2007 and 2008.





Number of patients and consumers involved in the European Medicines Agency activities 2007-2009



The evidence showed that, compared to 2007 and 2008 a more significant number of patients/consumers participated in the different activities of the Agency during 2009.

- Members:

The increased participation in the number of members is due to the new members and alternates for the CAT (which met for the first time in January 2009).

- Experts:

There have been 108 experts involved during 2009, compared to 87 in 2008 and 22 in 2007, which can mainly be attributed to:

  - Increased number of Package Leaflets and EPAR summaries sent out for review;
  - Participation in the training on the review of product information;
  - Involvement in the Working Group on clinical trials in third countries;
  - More participation in the activities of the Scientific Advice Working Party (3 times in 2009);
  - More participation in SAG meetings;
  - More participation in the preparation of safety communications (only 1 example in 2007, 3 in 2009);
  - Increased number of consultation requests from the PhVWP;

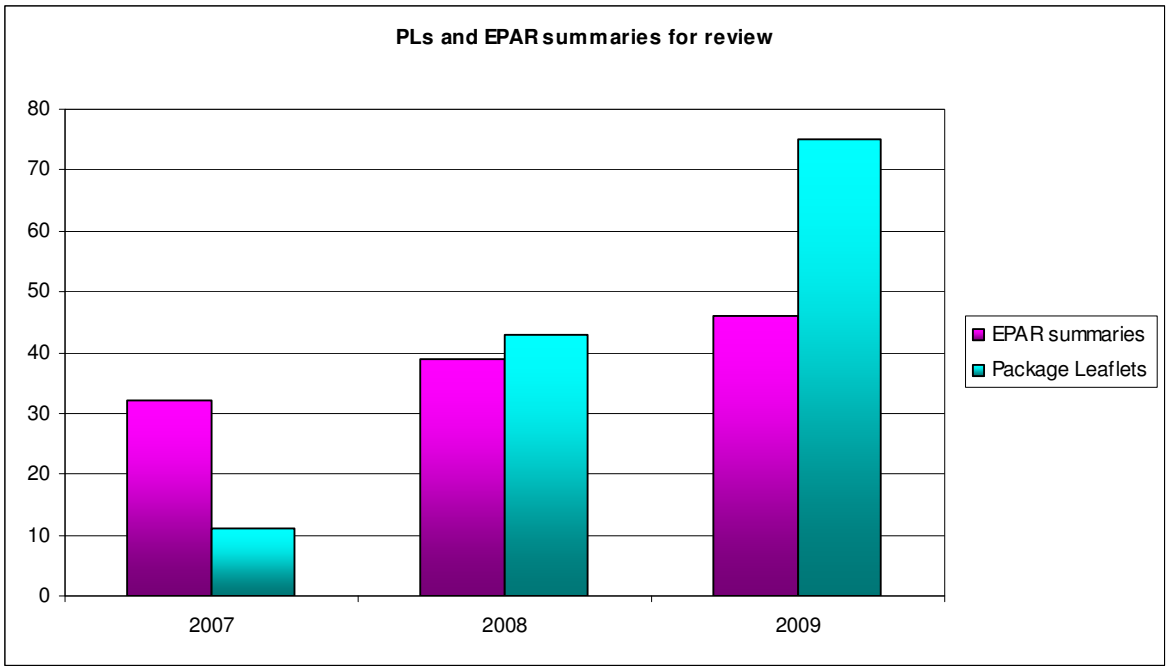
- Representatives:

There were 70 organisations' representatives involved during 2009, compared to 47 in 2008 and 28 in 2007, which can mainly be attributed to a more systematic participation of patients'/consumers' representatives in workshops and other events organised by the Agency:

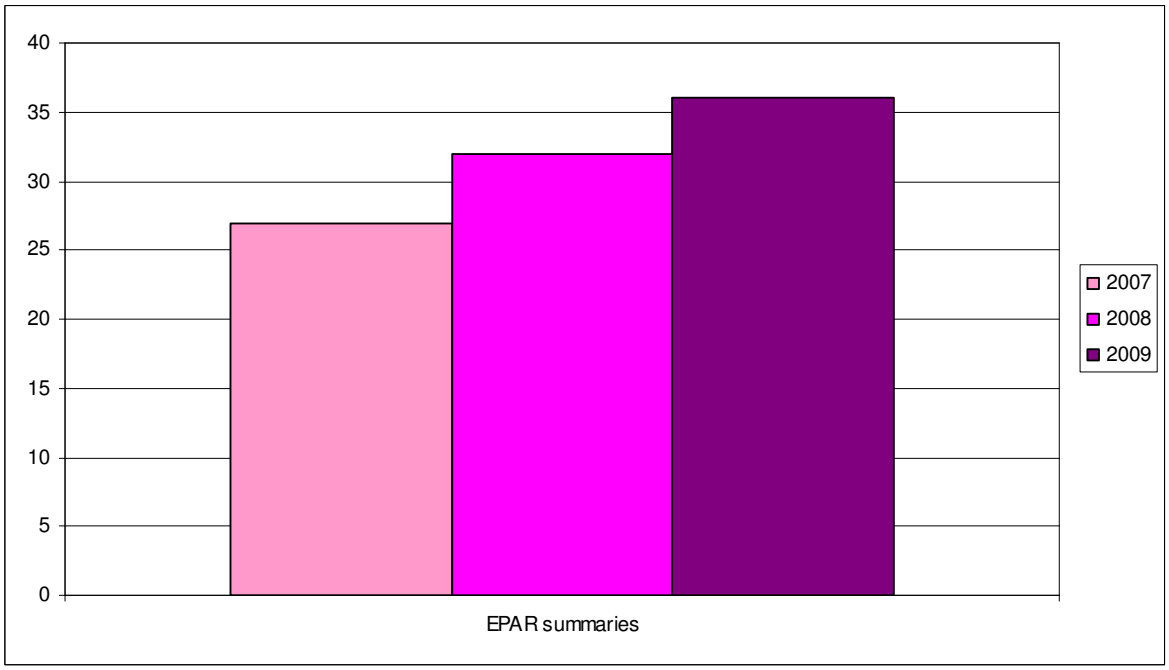
- EMA conferences and workshops (workshops on transparency, EudraCT information day, meeting with EC representatives to discuss the pharma-package);
- Consultation on the new website design;
- PhVWP and CHMP consultations;
- Pilot-phase observers at the PhVWP;

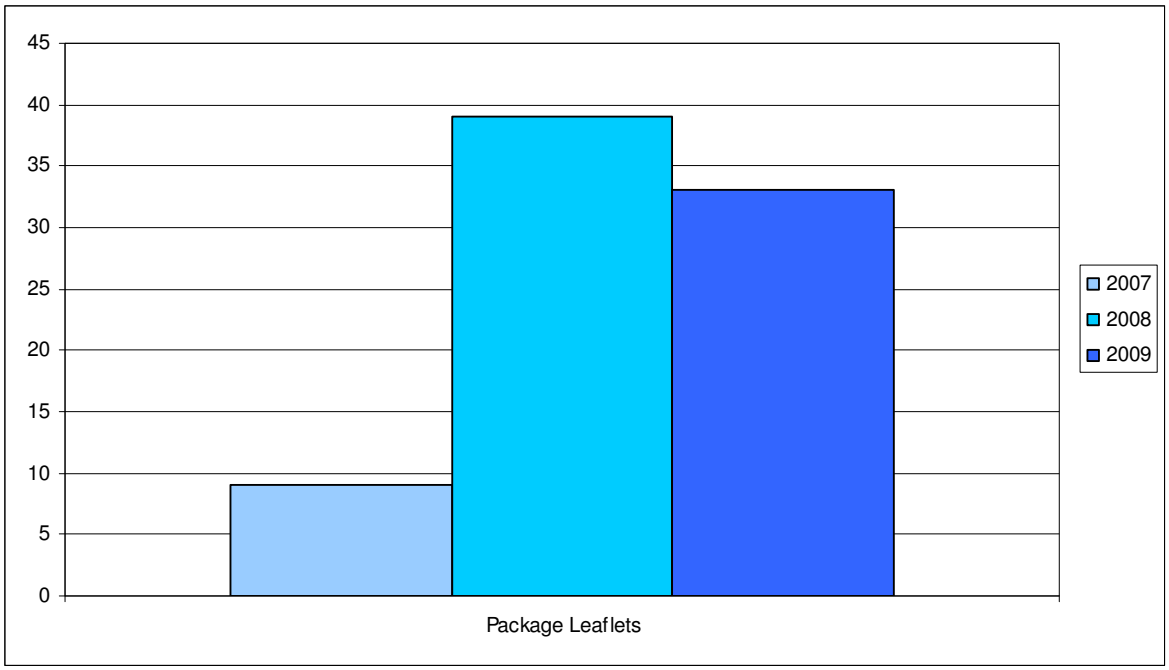
4.2. Document review procedure: comparative analysis of data from 2007, 2008 and 2009

A) The graph below shows the number of EPAR summaries and Package Leaflets sent out for review in 2009, compared to 2007 and 2008. The increase in the number of PLs sent out for review that is seen from 2008 is due to the extension of the scope of the procedure to cover also the PLs at the time of the initial authorisation (in 2007, PLs were sent for review only in case of renewal of the marketing authorisation).



B) The next two graphs below show the trend in the number of EPAR summaries and PLs actually reviewed, respectively, during the years 2007-2009.




45
40
35
30
25
20
15
10
5
0
2007
2008
2009
Package Leaflets

5. Conclusions and next steps

2009 has seen an important increase in the number of patients and consumers which have been involved in EMA activities (from **77** in 2007 to **165** in 2008, to **213** in **2009**). This is due to an increase in participation at all levels of the Agency's work (e.g. more members in scientific committees, more participation in Working Parties and Working Groups, more experts involved in scientific advice, more patients'/consumers' organisations consulted by the CHMP and by the PhVWP, involvement in Agency projects like ENCePP and PROTECT).

2009 has been the year of the preparation of the "Reflection paper on the further involvement of patients and consumers in EMA activities," which explores how to further develop this interaction in a more systematic way, and proposes specific actions for implementation. In the reflection paper, which has been endorsed by the EMA Management Board in December 2009, the Agency experience in involving patients and consumers in different areas has been analysed, and the added value of the participation of patients and consumers in the work of the Agency has been acknowledged and confirmed. This acknowledgment included the recognition of the efforts of the individuals as well as of the organisations in contributing to the Agency activities, and it translated into a proposal to provide financial support to patients/consumers in specifically defined circumstances.

The actions proposed in the past as a result of the analysis of the 2008 performance indicators have been implemented in their majority. The results of this analysis have been used to identify areas for improvement and to propose specific actions, which have been considered during the finalisation of the aforementioned "Reflection Paper". The implementation of these actions started in 2009 and will continue during 2010, as some of the actions have been incorporated in the PCWP Work Programme for 2010 and 2011.

One of the actions which have been proposed by PCOs in 2008 concerned the enlargement of the PCWP. Following this recommendation, and considering the need to involve more PCOs in the Agency activities in order to cover more therapeutic areas, the process for the enlargement of the PCWP has been initiated in 2009.

With regard to the provision of information, the Agency has continued implementing measures aimed at improving the quality of product related information adapted and oriented to patients and the general public. The initial scope of the review procedure has been extended during 2009, so that now the Agency involves PCOs representatives in the preparation of a wider range of documents

Next steps

- Following the endorsement by the Management Board of the proposals for action in the "Reflection Paper", implementation has started in January 2010.
- The "Framework of interaction" between the EMA and PCOs will be extensively revised during 2010 and 2011. A new set of performance indicators will be elaborated to measure the impact of the activities proposed in the reflection paper (including the provision of financial support to their participation).
- PCOs representatives will continue to be involved in the preparation of information oriented to patients and the general public. The procedures in place will be continuously monitored and reviewed whenever necessary. The Agency will ensure adequate support and training to those PCOs representatives involved in the review procedure.

- The Agency will also explore the best way to involve patients/consumers in the preparation of additional EMA information addressed to patients and the general public (i.e. emerging safety information) and the best way to ensure timely and effective communication of this information to the concerned European organisations.
- The Agency will provide further training in several areas (e.g. regulatory procedures, clinical trials, innovative medicines) which are considered of great interest by patients'/consumers' organisations.
- The Agency will explore how patients and consumers can be involved in the benefit/risk assessment of medicinal products, defining the criteria for their involvement. This will increase the possibility for more PCOs to participate in EMA activities.
- The role of patients and consumers in the different scientific committees of the Agency will also be defined.
- In June 2011 The Management Board will be presented with the report on the progress achieved in 2010.

6. Glossary of terms and abbreviations

Term or Abbreviation	Definition
AR (Assessment Report)	A report written by a regulatory agency about a regulatory submission
ATMP	Advanced Therapy Medicinal Product
BMWP	Similar Biological (Biosimilar) Medicinal Products Working Party
BPWP	Blood Products Working Party
BWP	Biologics Working Party
CAT	Committee for Advanced Therapies
CD	Commission Decision
Centralised Procedure	A Community registration procedure created by Council Regulation (EEC) No. 2309/93 and amended by Regulation 726/2004 for the authorisation of medicinal products, for which there is a single application, a single evaluation and a single authorisation allowing direct access to the single market of the European Community. The single scientific evaluation is made by a review team which is lead by a Rapporteur and Co-rapporteur (both CHMP members) on behalf of all EU Member States and is undertaken in a maximum of 210 days. The opinion of the CHMP is transmitted to the European Commission to be transformed into a single marketing authorisation applicable to the whole European Union. This procedure is compulsory for medicinal products derived from biotechnology, and in 6 specific therapeutic areas (products against HIV, cancer, neurodegenerative diseases, diabetes, auto-immune and other immune dysfunctions and viral diseases) and available at the request of companies for other innovative new products. Applications are submitted directly to the EMA.
CHMP	Committee for Medicinal Products for Human Use. A scientific committee responsible for formulating the opinion of the European Medicines Agency on any question concerning the evaluation of human medicinal products. The CHMP comprises one member (and an alternate) nominated by each European Member State on the basis of their knowledge and experience in medicinal product evaluation, and 5 co-opted additional members. The term of office is 3 years and is renewable. The CHMP may establish working parties, expert groups and scientific advisory groups. It forms Scientific Opinions on Centralised applications as well as developing and approving new regulatory guidelines and reviewing issues which go into Arbitration.
Clock stop	A period of time during which the 'clock' that calculates the length of a procedure 'stops' while the applicant prepares answers to questions asked by the regulatory authority, e.g. in the centralised procedure, the time between day 120, when the CHMP issues a List of Questions (LoQ), and day 121, when the applicant submits the answers
CMD(h)	Co-ordination group for Mutual Recognition and Decentralised Procedures (human)

COMP	Committee for Orphan Medicinal Products
CMS	Concerned Member State. See MR (Mutual Recognition) and Decentralised Procedures.
Competent Authority	An authority in a European Member State responsible for the authorisation and supervision of medicinal products. Often abbreviated as NCA (national competent authority).
Conditional approval	Medicinal products that meet unmet medical needs, and in the interests of public health, may be granted a marketing authorisation (MA) on the basis of less complete clinical data than is normally the case and subject to specific obligations ('conditional marketing authorisations'), see Regulation (EC) No 507/2006). The conditional MA is renewed on an annual basis. It becomes a normal MA once all Specific Obligations (SOs) have been fulfilled.
Co-rapporteur (Centralised Procedure)	A second member of Committee for Medicinal Products for Human Use (CHMP) contributing to the assessment of a Centralised Procedure application. The specific role of the co-rapporteur is defined on a case-by-case basis by the Committee.
CPWP	Cell-based Products Working Party
DCP	Decentralised Procedure. Procedure for authorisation of medicinal products as stated in Article 28(3) of Directive 2001/83/EC as amended. It applies to products for which the Centralised Procedure is not mandatory and which are not authorised in any EU member state. It facilitates the parallel submission to both Reference Member State (RMS) and Concerned Member States (CMS) with parallel granting of national Marketing Authorisations.
DDL (Dear Doctor Letter)	Dear doctor letters are the means for a MAH to communicate to physicians, important labelling change, generally safety-related. The content of the letter is negotiated by the pharmaceutical company with the Regulatory Authority. In EU legislation a DDL is normally known as a Direct Healthcare Professional Communications (DHPC).
Decision	An EU instrument which is binding on those to whom it is addressed, whether it be a company, a Member State, or several Member States. In the context of the European Centralised Procedure, the final Decision on whether a product is granted a marketing authorisation is made by the European Commission.
DHPC	Direct Healthcare Professional Communication. See 'DDL'
DoI	Declaration of Interests
EC	European Commission
EMA	European Medicines Agency (was EMEA until December 2009)
ENCePP	European Network of Centres for Pharmacoepidemiology and Pharmacovigilance
EPAR	European Public Assessment Report. The European Public Assessment Report is prepared by the EMA with the CHMP/CVMP members who evaluated the MAA. EPARs are prepared for all dossiers evaluated via the centralised Procedure in all cases where the CHMP/CVMP formulates positive final opinions.

	They are publicly available to third parties on request (and available via the Internet). They include the approved Summary of Product Characteristics plus information on labelling and package leaflets.
EU (European Union)	The Union of 27 member states: Austria, Belgium, Bulgaria, Czech Republic, Cyprus, Denmark, Estonia, Finland, France, Germany, Greece, Hungary, Irish Republic, Italy, Latvia, Lithuania, Luxembourg, Malta, Netherlands, Poland, Portugal, Romania, Spain, Slovak Republic, Slovenia, Sweden and United Kingdom
EudraCT	The EudraCT database provides information on clinical trials taking place in the Community, as required by the clinical trials directive (Directive 2001/20/EC). The sponsor of a trial with at least one clinical trial site in the Community must use the database to obtain a unique number, needed before they can apply for permission to conduct the trial (applications to the Ethics Committee and to the competent authority).
Eudralink	Application for secure message transfer and communications between EMA and its stakeholders
Eudranet	European Drug Regulatory Network
EWP	Efficacy Working Party
Generic medicinal product	A medicinal product which has the same qualitative and quantitative composition in active substances and the same pharmaceutical form as the reference medicinal product, and whose bioequivalence with the reference medicinal product has been demonstrated by appropriate bioavailability studies. (Reg. 726/2004, Art 10, 2b) Generic "copies" can only be marketed after the originator's patent protection and/or marketing exclusivity has expired.
GTWP	Gene Therapy Working Party
HCP	Health Care Professional. A doctor, dentist, nurse, pharmacist or registered ophthalmic optician or other officially registered health professional.
HCP WG	EMA/CHMP Working Group with Healthcare Professionals' Organisations
HMPC	Committee for Herbal Medicinal Products
MA	Marketing Authorisation
MAA	Marketing Authorisation Application
MAH	Marketing Authorisation Holder
Market Exclusivity	Orphan Drugs which have been designated as such in the EU also receive a 10-year market exclusivity for the indication for which they have been authorised
MB	Management Board
Medicinal Product	A finished dosage form, for example, tablet, capsule, solution, etc., that contains an active ingredient generally, but not necessarily, in association with inactive ingredients. The term also includes a finished dosage form that does not contain an active ingredient but is intended to be used as a placebo.
MR	Mutual Recognition. A community registration procedure described by Council Directives 2001/82/EC and 2001/83/EC (as amended) for the authorisation of

	medicinal products. The Mutual Recognition Procedure is one of the routes for seeking regulatory approval in the European Union. A submission is first made to an EU Member State authority which assesses, grants a national approval and prepares an assessment report. This report is circulated by the initial authority to the other (concerned) Member States who are expected to recognise this decision and grant their own national authorisation within a period of 90 days following the initial approval. The 90 day period is used to resolve any issues between Member States. If serious objections are raised then the application is referred to CHMP for arbitration leading to a binding decision. Concerned Member State: A Member State which is concerned (i.e. included in the mutual recognition phrase) with an application for Mutual Recognition, and expected to recognise the initial approval of the Reference Member State.
MS	Member State. A country which is a member of the European Union.
NCA	National Competent Authority. See competent authority
Orphan Drug	A drug for the treatment of a rare serious disease (defined in the EU as a condition that affects not more than 5 in 10,000 persons in the Community, and in the US as a condition affecting fewer than 200,000 people in the US) or for a disease not likely to generate sufficient profit to justify Research and Development (R&D) costs
OTC	Over-the-counter. Medicinal products which may be purchased without a doctor's prescription.
PCWP	EMA Human Scientific Committees' Working Party with Patients' and Consumers' Organisations. Human Committees are CAT, CHMP, COMP, HMPC and PDCO.
PDCO	Paediatric Committee
pdf	Portable Document Format
PgWP	Pharmacogenomics Working Party
PhVWP	Pharmacovigilance Working Party
PL	Package Leaflet
Placebo	An inert and innocuous substance used especially in controlled clinical trials which allow tests of the efficacy of another treatment
Q&A	Question and answer document
QRD	Quality Review of Documents
QRD Group	Working Group on Quality Review of Documents. Provides assistance to the EMA scientific committees and to companies on linguistic aspects of the product information (summary of product characteristics, labelling and package leaflet).
QWP	Joint CHMP/CVMP Quality Working Party
Rapporteur	In the Centralised Procedure, a member of the CHMP appointed to co-ordinate the evaluation of an application
Renewal	A report submitted to a European or International regulatory authority after the first 5 years of authorisation (for EU Mutual Recognition and Centralised Procedures) for every active marketing

	authorisation. After a renewal after 5 years, the CHMP will decide if a further renewal may be necessary.
Review Clock	Time frame for regulatory authority review. This time typically gets extended if the reviewer asks questions of the applicant/sponsor.
RMS	Reference Member State. The European Member State who conducts the primary review of the MAA in the Mutual Recognition/Decentralised procedure and whose Assessment Report is used as the basis for mutual recognition (see also CMS).
SA	Scientific Advice
SAG	Scientific Advisory Group
SAWP	Scientific Advice Working Party
SWP	Safety Working Party
VWP	Vaccine Working Party
WP	Working Party