The European Agency for the Evaluation of Medicinal Products
Pre-authorisation Evaluation of Medicines for Human Use

London, 16 September 2002
Doc. Ref: EMEA/CPMP/2457/02

REPORT FROM THE 1st EMEA/CPMP WORKSHOP WITH PATIENTS' ORGANISATIONS:
Information and participation

Meeting chaired by Dr. Brasseur, Chairman of the CPMP
EMEA, 31 May 2002

The EMEA with its Committee for Proprietary Medicinal Products (CPMP) organised a workshop with representatives of seven European patients' and consumers' organisations.
The objectives of this workshop were to reinforce communication with patients' representatives, to exchange views on information related to medicinal products and to further explore contributions to regulatory activities. The agenda and the list of participants can be found in Annexes 1 and 2 respectively.

Mr Thomas Lönngren, EMEA Executive Director, welcomed the participants, emphasizing the importance of this meeting as an additional initiative to improve EMEA transparency. The EMEA already hosted the 1st Workshop for Patients' Organisations on Orphan Medicinal Products in March 2001. Dialogue with interested parties started since the EMEA was created in 1995, but the need for new ways of interacting with patients' organisations has been recognised. The contribution of the patients in the management and rational use of medicinal products is becoming increasingly common and therefore information to patients and participation to regulatory activities should be improved. Information provided to patients is a priority for the EMEA and is also an important aspect of the current review of the Community legislation as recently highlighted by the High Level Group on innovation and provision of medicines - G10 Medicines group. The importance of the meeting was stressed as a unique forum for communication to the patients of the activities of EMEA/CPMP and for exchange of views and ideas on how to improve the contribution of patients in these activities.

Dr Brasseur, Chairman of the CPMP, opened the morning session devoted to a general overview of the EMEA activities and its transparency policy, highlighting the recent changes in interactions and expectations between the authorities, pharmaceutical companies and patients. These changes were made possible as better information was provided to patients and public had a better comprehension of medicines (presentation 1).
An introduction to the EMEA, its role and structure was provided by Dr Le Courtois (presentation 2). The EMEA, a networking decentralised agency created in 1995, is the result of 30 years' harmonisation in the field of medicinal products in Europe. Its rapid and successful development will allow the EMEA to face the challenge of the new EU legislation as well as political changes such as the enlargement, which will have major consequences on the EMEA activities.
Since its creation, the EMEA has put in place a transparency policy (presentation 3 by Mr Harvey). As part of the EU Institutional framework, the EMEA is bound by clear transparency obligations under the EU Treaty, under the scrutiny of the European Ombudsman. The Agency has been keen from the outset to establish and continuously improve high transparency standards, namely by extending the scope of regulatory information available to the public and increasing the interaction with interested parties. It was announced that the Agency would be making proposals later in the year for new transparency initiatives. In addition to the proposals made in the context of the EU 2001 Review, which could have implications on the transparency policy, the importance for patients' organisations to give feedback on their needs and expectations in terms of information available was highlighted.

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In the subsequent discussion, the status of the Agency and its independence from the European Commission also raised questions since the responsibility of granting the marketing authorisation remains with the European Commission, which is also responsible for the legislation of the pharmaceuticals. As an example was mentioned the current legislative framework which only allows the Agency to publish European public assessment reports (EPARs) for medicinal products for which a marketing authorisation was granted. The Agency's scientific independence was not questioned. The transparency policy raised questions, particularly on the publication of EPARs for negative opinions. The Agency took however a further step in 2002 by publishing Summary of Opinions for both positive and negative opinions adopted by the CPMP.

The second session of the morning, chaired by Prof Bass focused on the CPMP activities and network. The CPMP issues its scientific opinion based on quality, efficacy and safety criteria.
The critical steps of the life of a medicinal product were reviewed, insisting on the need for a proper development in order to demonstrate the efficacy of the product and to exclude any major safety problem incompatible with a safe use of the product (presentation 4 by Dr Lekkerkerker). Based on these studies, the regulatory agencies make their assessment and define the benefit/risk ratio of the product, which has to be positive for approval. The key concept of the evaluation was illustrated with some examples showing the impact on public health and the implication on the product information, i.e Summary of Product Characteristics, labelling and package leaflet.
Once a product has been authorised, the post-marketing surveillance starts (presentation 5 by Dr. Bahri). The EMEA coordinates a network involving different partners i.e, marketing authorisation holders, Member States, the European Commission and health care professionals who report adverse drug reactions they observe with medicinal products. The EU pharmacovigilance system involves all products authorised in the EU. The participants raised a number of questions on the pharmacovigilance system and made comments such as the need to make patients aware of what pharmacovigilance is, its relevance and how to increase the reporting of adverse reactions. The participants suggested also the possibility to explore other ways of communicating to the public safety information based on the experience gathered with other medicinal products e.g oral contraceptives.

Information available to the patient was the theme of the next session, chaired by Mr Wathion.
The Agency makes publicly available European public assessment reports (EPARs) for all products authorised after evaluation by the Agency (presentation 6 by Mr Wathion). In addition, the Agency releases a summary for all CPMP opinions, either positive or negative, on the granting of a marketing authorisation for initial applications.
Achieving a high quality of the product information in all EU languages is a concern for the Agency and therefore initiatives have been taken to improve the quality in terms of readability, particularly of the package leaflet. For instance, linguistic review groups, templates and guidance documents have been created (presentation 7 by Mrs Boone). Experience with readability/user testing of package leaflets is still limited, however it is anticipated that results will highlight areas for improvement on the package leaflet.
During the discussion, participants recognised the package leaflet as a useful information tool for patients, and further reflection on how to improve it will be necessary. One suggestion made was to involve patients in the review process of the package leaflet. The Commission has also acknowledged the problem as part of the EU 2001 Review. This was further reflected in the recommendations elaborated by the G10 Medicines group.
The proposed amendments to the pharmaceutical legislation in the context of EU 2001 Review were highlighted (presentation 8 by Dr Santos Ivo, on behalf of Dr North of the European Commission). Although there are no major changes to the fundamental principles, proposals have been made, among others on procedural aspects to increase the availability of new medicinal products such as fast track procedures, particular marketing authorisations, compassionate use, shortening of the decision-making phase. In addition it is proposed to strengthen pharmacovigilance requirements and increase transparency to public.
As part of its transparency policy, the EMEA created its website in 1995 aiming at providing both medical, scientific and regulatory information to patients/health care professionals, pharmaceutical companies, other regulatory authorities and press orientated information (presentation 9 by Dr Moulon and Mr Ettore). The web is an important communication tool to widely disseminate information and it is recognised that there is room for improvement. Some problems have already been identified such as

the need to highlight the information addressed to patients and the accessibility to people with disabilities. It is important however to receive feedback from users/patients to further improve the quality of the EMEA website.

Mrs Greene chaired the afternoon session dedicated to patients associations: needs and expectations. She reminded participants that patient organisations have also a responsibility in providing adequate information to patients.
The values that should govern associations were discussed such as transparency, representativeness and consultation (presentation 10 by Mr Hayes). The associations, which are also information provider, have to work in a transparent, honest and ethical way and should take into account the internal market implication: equal standards for everybody everywhere in the EU. Beyond the information made available by the associations, the role played by the physician and pharmacist is also essential in providing adequate explanation to allow the patient informed choice, taking into account that patients are often in vulnerable situations when receiving the information.
Another controversial area discussed was pharmacovigilance and the problem of under-reporting of adverse reactions (presentation 11 by Mrs Villa). The proposals foreseen in EU pharmaceutical legislation to strengthen pharmacovigilance requirements were considered to be insufficient to increase the reporting. In this context, further improvements are needed for instance providing feedback on the reports and increase access to data. There is also a need to further "educate" patients and health professionals on pharmacovigilance issues and their importance. It is recognised that only 10 % of prescribers have to report adverse reactions to provide sufficient data for regulators to take necessary actions.
The following discussion questioned the desirability for direct reporting from patients. BEUC considers direct reporting like in the USA and Canada as a first step to increase reporting even though there is a risk for a potential overload of the system and the consequent difficulties to validate the information. The participants considered this proposal interesting especially in the case of orphan medicinal products. However it was recognised that the collaboration between prescriber and patient should be improved to ensure an adequate reporting.
An example of fruitful participation of a patient association in drug development was presented (presentation 12 by Mr Houÿez). EATG has initiated collaboration with the pharmaceutical companies to the clinical development of some anti-HIV medicinal products identified as potentially important for the community and in some cases succeeded to accelerate the set up of the expanded access programmes. To achieve this, the group interacts with the scientific advisory board of the company and asks the opinion of independent investigators on both the product and the development programme. In addition relations with the CPMP have developed over time: for instance the group has met with the CPMP to discuss specific issues or to communicate concerns on drug evaluation/approval.
The proposals made in the context of the EU 2001 Review were discussed from the perspective of a patient particularly the faster access to highly innovative medicines, new timeframes and direct to consumer information (presentation 13 by Mr Van der Zeidjen). Although the initiatives on the fast track procedure, conditional marketing authorisation and compassionate use are in principle welcomed, reservations were made on how they would work in practice and whether they would help access to new innovations for patients with fatal medical condition without any treatment alternatives, even if the efficacy/safety of these products are not fully established. Reservations were also expressed on the proposal for direct to consumer information, which is one of the most controversial proposals from the Commission according to the participants. It is recognised that patients need to obtain independent information to improve communication with their doctors, but whether pharmaceutical companies should generate this information is questioned. In the proposal, the direct to consumer information focuses on three diseases for the duration of a pilot phase. Patient associations have questioned the choice of these diseases.
Preliminary results of a survey carried out by IAPO on the views of EU based patient groups on the direct to consumer information were presented (presentation 14 by Mrs Wyke). The results confirm that patients wish to obtain information about authorised prescription medicines since 50 % of the patients' organisations participating in the survey were favourable to direct to consumer information. However 17 % indicated that strict limitations would be necessary to prevent any risk of advertising. In addition, according to the survey, most of them were of the opinion that this information should

reach the patient via the physician and patient group. The survey highlighted also the deficiencies of the package leaflet since less than half of the patients' organisations trusted their content.
The positive experience of patients' organisations participation to regulatory activities for orphan medicinal products was finally presented (presentation 15 by Mr Le Cam). The scientific committee responsible for examining all applications for orphan medicinal product designation (COMP), which first met on 17 April 2000, comprises three patient organisation representatives. In addition patient organisations participate in workshops and working groups set up in the context of orphan drugs. How this experience could be expanded beyond the orphans to allow a greater involvement to the CPMP activities was discussed. The following suggestions were made: organisation of regular workshops, proactive consultation of concerned patient groups on guidance documents, provision of external expertise to CPMP and its satellites groups on medicinal products. The need for the EMEA to use additional communication tools than the website to reach patient organisations was also highlighted.

The discussion focused on the importance of providing information adapted to patients' needs, developing appropriate communication tools, and increasing the awareness of the public in relation to the use of medicinal products, particularly in the context of pharmacovigilance. The following suggestions were made:

- Improve the readability and structure of the package leaflet;
- As the current structure of the EPAR was recognised too scientific for patients, a more readable document could be sent at the time of marketing authorisation to patient organisations to further dispatch it to patients. This document should be available in all different languages.
- Increase awareness of the EMEA and its activities by publishing editorials, articles in patient organisations' newsletters;
- Include a link to the EMEA web site on patient organisations' websites;
- Identify the right partners and links;
- Reflect on new measures for further transparency;
- Educate patients and health professionals on pharmacovigilance and its relevance to increase the reporting of adverse events.

Dr Le Courtois concluded that although it is a long process, dialogue and cooperation should start with a pragmatic approach. The following way forward was therefore agreed upon:

- A small working group will be set up within the Agency in order to make proposals for action on the identified areas.
- Within a year, a 2nd workshop should be organised where the progress of the working group will be reported. This 2nd workshop could involve additional patient organisations to expand the platform.

Dr Brasseur thanked the participants for the excellent contributions as the meeting has met the objectives and had provided a fruitful opportunity to exchange information, views and ideas.
The participants were informed that a press release of the meeting will be published on the EMEA website followed by a more extensive report of the meeting with in annexes the slides of the presentations.

ANNEX 1

MEETING AGENDA

The European Agency for the Evaluation of Medicinal Products
Pre-authorisation Evaluation of Medicines for Human Use

London, 29 May 2002
Doc. Ref: EMEA/6155/02

1st EMEA/CPMP Workshop with Patients' Organisations :
Information and participation

31 May 2002, 10.00-16.00
EMEA 4th floor, Conference Room A

AGENDA

Chairperson: Daniel Brasseur

~ Registration + Coffee: 9.30-10.00 ~

WELCOME BY MR. THOMAS LÖNNGREN, EXECUTIVE DIRECTOR OF EMEA

1.	**Introduction**		**10:00-10.30**
	1.1	**Introduction to the Workshop** Daniel Brasseur, Chairman of CPMP	*10 mins*
	1.2	**Introduction to the EMEA** Patrick Le Courtois, Head of Unit, EMEA	*10 mins*
	1.3	**Transparency Policy – The way forward** Martin Harvey, EMEA Directorate	*10 mins*
2.	**CPMP / EMEA Activities and Network** *(Chaired by Rolf Bass, CPMP Member)*		**10:30-11:20**
	2.1	**Key concept of the Evaluation and impacts on public health** Fritz Lekkerkerker, CPMP Member	*15 mins*
	2.2	**Pharmacovigilance - Post-marketing surveillance** Priya Bahri, EMEA	*15 mins*
	2.3	*Questions and Discussion*	*20 mins*

~ Coffee break: 11.20-11.35 ~

3.	**Information available to the patient** *(Chaired by Noel Wathion, Head of Unit, EMEA)*		**11:35-12:30**
	3.1	**Summary of Opinions and EPARs, Summary of Product Characteristics and Package Leaflet readability** Hilde Boone, EMEA	*15 mins*
	3.2	**Proposals in the European Commission Review** Arielle North, DG Enterprise, European Commission	*10 mins*
	3.3	**Website Presentation** Isabelle Moulon & Riccardo Ettore, EMEA	*15 mins*
	3.4	*Questions and Discussion*	*15 mins*

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~ Lunch in EMEA's Self-Service Restaurant: 12.30-13.30 ~.

4.	**Patients Associations: Needs and expectations** *(Chaired by Lesley Greene, EURORDIS (CLIMB) UK)*		**13:30-16:00**
	4.1	**Patient empowerment : towards informed choice** Andrew Hayes, EPHA	*15 mins*
	4.2	**Pharmacovigilance: who knows about it - a point of view from the consumers** Louisa Villa, BEUC	*15 mins*
	4.3	**Involvement in drug development** Francois Houyez, EATG	*15 mins*
	4.4	**Review 2001 : patients point of view** Albert van der Zeijden, IAPO	*15 mins*
	4.5	**Patients' Organisations Participation: an added value for the regulatory system** Yann Le Cam, EURORDIS	*15 mins*
5	**Discussion and Proposals**		*60 mins*

~ Closing Remarks by CPMP Chairperson and EMEA ~

ANNEX 2

PARTICIPANTS LIST

The European Agency for the Evaluation of Medicinal Products
Pre-authorisation Evaluation of Medicines for Human Use

London, 6 June 2002
EMEA/13971/02

1st EMEA/CPMP Workshop with Patients' Organisations: Information and participation

PARTICIPANTS LIST

ORGANISATION	NAME
BEUC: Bureau Europeen des Unions de Consommateurs	Charlotte de Roo (Belgium) Louisa Villa (Italy)
EATG: European Aids Treatment Group	Mauro Guarinieri (Italy) François Houÿez (France) Jorma Koskinen (Finland)
EFNA: European Foundation of Neurological Association	Alistair Newton (UK) Evelyn Sipido (Italy)
EPHA: European Public Health Alliance	Andrew Hayes (UK) Emmanuel Trenado (France) Colin Webb (UK)
European Cancer Leagues	Kate Law (UK) Philippe Mourouga (France) Richard Sullivan (UK)
EURORDIS: European Organisation of Rare Disorders	Fabrizia Bignami (France) Yann le Cam (France) Lesley Greene (UK) Françoise Salama (France)
IAPO: International Alliance of Patient Associations	Albert van der Zeijden (Netherlands) Alex Wyke (UK)
CPMP Members	Daniel Brasseur – CPMP Chairman Rolf Bass Manfred Haase Fritz Lekkerkerker David Lyons Cristina Sampaio Nathalie Morgensztejn (CPMP expert)

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EMEA	Thomas Lönngren - EMEA Executive Director Priya Bahri Hilde Boone Riccardo Ettore Martin Harvey Patrick Le Courtois Isabelle Moulon Marisa Papaluca-Amati Rui Santos-Ivo Nathalie Seigneuret Noel Wathion