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

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COMP/34/02

PRESS RELEASE

1st EMEA Workshop with Health Professionals and Academia on Orphan Medicinal Products

On 24 January 2002, the EMEA hosted the 1st Workshop for Health Professionals and Academia on Orphan Medicinal Products. Seventeen representatives of Learned Societies and other Medical Associations from across the European Union met with members of the Committee for Orphan Medicinal Products (COMP), and representatives of the EMEA. The list of participants is provided in Annex.

Prof. Torrent-Farnell (Chairman of the COMP) and Dr Le Courtois (Head of the Pre-authorisation Unit for the Evaluation of Medicinal Products for Human Use), co-chairs of the meeting, welcomed the participants and emphasised the orphan initiative as a priority for the EMEA and the importance of working closely together with Health Professionals, Academia and National Organisations funding research.

In follow up to the workshops held with patient organisations and pharmaceutical industry last year, this was the third meeting with interested parties organised by the EMEA on Orphan Medicinal Products.

The objectives of this workshop were to establish communication links with representatives of Health Professionals and Academia, to share information on EMEA activities, to exchange views and expectations on development and access to orphan medicinal products and to explore possibilities for benefiting from the expertise of the Learned Societies and other Medical Associations.

The first session on "The role of the EMEA and orphan medicinal products" was moderated by Dr. Harrie Seeverens, COMP member from the Netherlands. The presentations included an overview of the Scientific Committees of the EMEA and of the role of experts in particular in the evaluation of orphan drug designation applications and in the provision of Protocol Assistance for the development of orphan medicinal products. Furthermore an overview of COMP activities to date was presented including its impact on the availability of orphan medicinal products for patients suffering from orphan conditions.

It was emphasised that in addition to the designation of orphan medicinal products one of the tasks of the COMP was to advise the European Commission on all issues associated with orphan medicinal products. In this context aspects related to differentiated access for patients to authorised orphan medicinal products which results from national policies will be raised with the Commission.

The second session, "Building a partnership with patients and health professionals/academia", moderated by Dr. Kerstin Westermark, COMP member from Sweden, included the following topics: the role of academia/health professionals in orphan medicinal product development; a patient representative's and health professional's view of the orphan initiatives; a case study of the designation procedure from a health professional and start-up company's view point.

It was concluded that with a view to increasing communication, emphasis should be put on the following aspects in the near future:

- In view of new emerging therapies and of the methodological difficulties for clinical trials resulting from the small patient populations the COMP/EMEA will explore the possibility of routinely utilising the expertise of Learned Societies and other Medical Associations in orphan designation and protocol assistance
- The COMP/EMEA welcomed the proposal to participate in meetings organised by the invited associations to present its activities in the field of orphan medicinal product.

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The EMEA will continue to develop as a platform for communication with all parties involved in orphan medicinal products. Links with the European Commission's Directorates and national bodies responsible for public health and research initiatives into rare diseases will be reinforced and a meeting with all interested parties, patient organizations, pharmaceutical industry and health professionals and academia is foreseen to be organised in the second half of 2002.

NOTE: This Press Release, together with other information about the work of the EMEA, may be found on the internet at the following location: http://www.emea.eu.int/

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ANNEX

List of Participants
1st EMEA Workshop with Health Professionals and Academia on Orphan Medicinal Products

held on 24 January 2002

Co-chairmen:

Prof. Josep TORRENT-FARNELL	COMP Chairperson
Dr. Patrick LE COURTOIS	EMEA, Head of Pre-authorisation Unit

Health Professionals and Academia:

Prof. Helga M ÖGMUNDSDOTTIR	European Association for Cancer Research
Dr. Gérard PONS	European Association for Clinical Pharmacology and Therapeutics
Mr Archie TURNBULL	European Respiratory Society
Prof. George DICKSON	European Society of Gene Therapy
Prof. Heine H. HANSEN	European Society for Medical Oncology
Prof. Ragnar NORRBY	European Society of Clinical Microbiology and Infectious Diseases
Dr. Ségolène AYMÉ	European Society for Human Genetics
Dr. Cillian TWOMEY	European Union of Medical Specialists
Dr. Leonard P. HARVEY	Standing Committee of European Doctors
Prof. Ruth MATTHEWS	*Neu*Tec Pharma plc
Prof. Janet DARBYSHIRE	Medical Research Council
Dr. Joe MCNAMARA	Medical Research Council
Dr. John WALTER	Society for the Study of Inborn Errors of Metabolism
Dr. Edvard BEEM	Netherlands Organisation for Health Research and Development
Dr. Paola BAIARDI	Italian Task Force Orphan Drugs/Rare Diseases
Dr. Carole MOQUIN-PATTEY	INSERM
Mr François SCHILTZ	Engelhorn Foundation For Rare Diseases

COMP Members:

Dr. François MEYER	France
Dr. Domenica TARUSCIO	Italy
Prof. Henri METZ	Luxembourg
Dr. Harrie J.J. SEEVERENS	The Nederlands
Dr. Kerstin WESTERMARK	Sweden
Mr. Yann LE CAM	COMP Vice-Chairperson, Representative of Patient Organisation
Mr. Alastair KENT	Representative of Patient Organisation
Dr. Randi NORDAL	The Norwegian Medicines Control Authority - Observer
Prof. Gianmartino BENZI	EMEA Representative
Dr. David LYONS	EMEA Representative

EMEA Secretariat

Dr. Spiros VAMVAKAS
Ms Melanie CARR
Dr. Driss BERDAI
Mr Marco PITRONE
Ms Johanna NORDLUND

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