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

25 April 2001
COMP/141/01

PRESS RELEASE

First EMEA Workshop with Industry on Orphan Medicinal Products

On 11 April 2001, the EMEA hosted the first workshop with Pharmaceutical Industry on Orphan Medicinal Products. Representatives of the Pharmaceutical Industry from across the European Union met with members of the Committee for Orphan Medicinal Products (COMP), the European Commission and representatives of the EMEA. Since EU Legislation[1] on orphan medicinal products was implemented, 34 orphan medicinal products have already been designated in the European Union and 103 further applications have been submitted or announced. As one year has passed since the first meeting of the COMP took place, it was felt important to share experience and to discuss future developments in transparency with sponsors of designated orphan medicinal products and trade associations represented by EBE (Emerging Biopharmaceutical Enterprises, a specialised group of EFPIA, the European Federation of Pharmaceutical Industries and Associations) and EuropaBio (The European Association for Bioindustries).

The workshop was introduced by Dr Patrick Le Courtois (Head of Pre-Authorisation Human Medicines Unit, EMEA) and co-chaired by Prof Josep Torrent-Farnell (COMP Chairman) and Mr Brian Ager (EFPIA, Director General).

The novelty of the procedures put in place since the introduction of the EU orphan legislation and the specific role and competence of the COMP were highlighted, in particular with regard to patients' representation in the Committee and the involvement of the EMEA in preparation of the work of the Committee. It was underlined that the challenges faced in terms of short evaluation timeframe and high number of applications should not jeopardize the consistency of the Committee's scientific evaluation for the designation of Orphan Medicinal Products. The COMP Chairman explained the importance of team work between COMP and EMEA and of the links between the COMP and the CPMP, particularly in relation to Protocol Assistance activities.

The importance of early consultation with the EMEA through pre-submission meetings was emphasized. Presubmission meetings facilitate processing of orphan designation applications and potential sponsors have been strongly encouraged to request them. Industry thanked the EMEA for its high level of accessibility and availability.

Industry stressed the importance of Protocol Assistance activities in optimising the development programme and the time to marketing authorisation. The importance of external experts involvement in orphan designation evaluation procedures was acknowledged and should be encouraged for Protocol Assistance.

Patients' associations and the Pharmaceutical Industry reiterated the importance of adequate funding by the European Commission of EMEA in order to make the EU regulation on Orphan Medicinal Products more effective. Funding from the European Commission supports EMEA activities and permits fee exemptions to be granted for Orphan Medicinal Products.

With a view to increasing EMEA transparency on Orphan Medicinal Products and COMP activities and taking note of the crucial and increasing role of the EMEA as a platform for communication between all interested parties, the participants agreed that the following proposals will be further developped:

[1] Regulation (EC) No 141/2000 adopted of 16 December 1999 and Commission Regulation (EC) No 847/2000 of 27 April 2000

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- Improvement of access to EMEA documents on Orphan Medicinal Products through a dedicated area on its website.
- Provision of a public summary report for positive and negative opinions on designations to be published by the EMEA at the time of decision.
- Preparation of additional EMEA/COMP guidelines aimed at improving and streamlining the content of applications for designation (e.g. on prevalence in rare diseases).
- Increase of opportunities for oral explanations.
- Establishment of performance indicators on orphan designation procedure.

As a medium term objective, the EMEA will explore the possibility of implementing a dynamic tool to allow EMEA and sponsors to disseminate updated data on designated Orphan Medicinal Products for access by the public.

All participants expressed interest in continuing dialogue on COMP activities. Industry representatives agreed on their participation to the implementation of dedicated working groups and to assist in the organisation of future technical workshops with other interested parties.

The list of participants is provided in Annex.

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
First EMEA Workshop with Industry on Orphan Medicinal Products

held on 11 April 2001

Chair:

Prof Josep TORRENT-FARNELL	COMP Chairman
Mr Brian AGER	EFPIA European Federation of Pharmaceutical Industries and Associations Director General
Dr Patrick LE COURTOIS	Head of Pre-Authorisation Human Medicines Unit, EMEA

Speakers:

Mr Yann LE CAM	COMP Vice-Chairman
Prof Dr Hans Georg EICHLER	COMP Member
Dr Francois MEYER	COMP Member
Dr Rick J LILLEY	Shire Pharmaceutical Development Ltd
Dr Paola RICCI	EFPIA/EBE & EuropaBio
Dr Behruz ESLAMI	EFPIA/EBE & EuropaBio
Dr Patrick LE COURTOIS	Head of Pre-Authorisation Human Medicines Unit, EMEA
Pharm. Noël WATHION	Head of Post-Authorisation Human Medicines Unit, EMEA
Dr Driss BERDAI	Scientific Administrator, EMEA

Participants:

Mrs Petra BADDACK	EFPIA/EBE
Ms Sue BARROWCLIFFE	EFPIA/EBE
Dr David BILL	EFPIA/EBE
Dr Erwin BOEHM	EFPIA/EBE
Dr Franz BUCHHOLZER	EFPIA/EBE
Mr Didier CAIZERGUES	EFPIA/EBE
Mrs Anne-Thérèse CREBASSA	EFPIA/EBE
Mrs Hanne DAMGAARD JENSEN	EFPIA/EBE
Mr Bertrand FOURNIER	EFPIA/EBE
Mr Adrianus FRUIJTIER	EFPIA/EBE
Mrs Anne-Marie GEORGES	EFPIA/EBE
Dr Gudrun HORNQUIST	EFPIA/EBE
Mrs Anne LEHERISSEL	EFPIA/EBE
Mr Alan MORRISON	EFPIA/EBE
Dr Maj-Inger NILSSON	EFPIA/EBE
Mrs Anne PAPIN DI POMPÉO	EFPIA/EBE
Mrs Lillan REJKJÆR	EFPIA/EBE
Mrs Susanne TRAENKLE	EFPIA/EBE
Mr Didier WOLF	EFPIA/EBE
Dr Karl BIRTHISTLE	EuropaBio
Dr Sergio DOMPE	EuropaBio
Mrs Marie-Christine FORTUN	EuropaBio
Mrs Jenny GREENHORN	EuropaBio
Mr William GUNNARSSON	EuropaBio
Mr Jean-Claude HAVAUX	EuropaBio
Ms Nathalie MOLL	EuropaBio
Mr Hugo SCHEPENS	EuropaBio
Mrs Carol SUMMERS	EuropaBio
Mr Andrea RAPPAGLIOSI	EFPIA/EBE & EuropaBio
Dr Erik TAMBUYZER	EFPIA/EBE & EuropaBio
Dr Gary ACTON	Cell Therapeutics (UK) Ltd
Dr M BACCOUCHE	Byk Gulden Pharmaceuticals

Mr David BOAL	Laxdale Ltd
Ms Margaret CROS	Sanofi-Synthélabo
Dr Petra DÖRR	ICN Pharmaceuticals Limited
Dr Peter F HEINZEL	Euro Nippon Kayaku GmbH
Dr Peter HERRMANN	Actelion Pharmaceuticals Ltd
Dr Robert M IBBOTSON	Oxford GlycoSciences (UK) Ltd
Ms Karen LEYSHON	Morgan, Lewis & Bockius
Dr Axel OLIVAR	Schering AG
Ms Stéphanie POTIER	Opi Orphan Pharma international
Dr Dominique PRADEAU	Pharmacie Centrale des Hôpitaux de Paris
Dr Bernard SONET	Laboratories SMB SA
Dr Emmanuelle VOISIN	Voisin Consulting SARL

COMP Members:

Mr Abascal ALONSO
Prof Gianmartino BENZI
Dr Rembert ELBERS
Dr Kalle HOPPU
Dr David LYONS
Dr H.J.J. SEEVERENS

European Commission:

Ms Emer COOKE	DG Enterprise

EMEA Secretariat:

Dr Spiros VAMVAKAS
Ms Melanie CARR
Dr Francesco PIGNATTI
Ms Theresa MC FADDEN
Ms Nicola MARTIN

Other

Ms Hawa DRAME SOW	Patients' Representative Support

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