European Medicines Agency
Pre-authorisation Evaluation of Medicines for Human Use

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PRESS RELEASE

2nd EMEA Workshop on Orphan Medicinal Products for Patients' Representatives and Learned Societies

On 4 March 2005, the EMEA hosted the 2nd Orphan Medicinal Product Workshop for Patients' Representatives and Learned Societies. Seven representatives of Patients' Organisations and twelve representatives of Learned Societies met with members of the Committee for Orphan Medicinal Products (COMP), the European Commission and representatives of the EMEA. Prof. Josep Torrent-Farnell (Chairman of the COMP) and Dr. Peter Arlett (European Commission's DG Enterprise) co-chaired the meeting. The list of participants is provided in Annex I.

As the EMEA approaches the 5th anniversary of the EU Orphan initiative, this meeting was organised to provide an overview of what has been achieved since the end of April 2000 when EU 'orphan' legislation[1] entered into force and to propose a forum for discussion on the experience acquired to date. The meeting was designed to facilitate the exchange of views and expectations on development and access to orphan medicinal products

In June 2005 the COMP will report to the European Commission on the experience acquired as a result of the application of the Orphan Regulation and the public health benefits that have been obtained. To prepare for this report, the views of Patients' Representatives and Learned Societies are of utmost importance. The COMP report will feed into the upcoming European Commission review of the Orphan Regulation in January 2006.

The workshop was introduced by Prof. Spiros Vamvakas (Acting Head of Sector for Scientific Advice and Orphan Drugs at the EMEA). The importance of close collaboration with Patients' Organisations and Learned Societies to the continued success of the orphan initiative was emphasised. Dr. Peter Arlett (European Commission's DG Enterprise) presented an overview of the EU Orphan legislation and Ms. Melanie Carr (Scientific Administrator, EMEA) highlighted some of the major milestones and achievements.

Mr. Alastair Kent (COMP Member representing patients' organisations and Director of European Genetic Alliances Network) introduced some of the key issues from the patient's perspective. Although the success of the COMP in encouraging development of orphan medicinal products to the same standards of quality, safety and efficacy as any other product was acknowledged, the problem of patient access to these new treatments was underlined. The need for the development of better models to assess clinical effectiveness of orphan medicinal products was also emphasised.

Prof. Bruce Morland (Member of the COMP Working Group with Interested Parties and Paediatric Oncologist at Birmingham Children's Hospital) highlighted the need to open up a dialogue with Learned Societies to achieve a greater understanding of the orphan legislation and to promote research into rare diseases and orphan medicinal product development.

In the afternoon two parallel break-out sessions with representatives of patient organisations and representatives of learned societies were held to permit focused discussion on issues relevant to both parties. Each group was invited to present their experience and voice any concerns. The final session concluded with a general discussion on future proposals.

It was agreed that the following proposals will be taken forward by the EMEA for further consideration:

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

7 Westferry Circus, Canary Wharf, London, E14 4HB, UK
Tel. (44-20) 74 18 84 00 Fax (44-20) 74 18 86 13
E-mail: mail@emea.eu.int http://www.emea.eu.int

- to improve communication, and provide more targeted and expanded access to information on orphan medicinal products
- to implement additional measures to facilitate clinical trials in rare diseases such as expert networking, guidance, etc.
- to increase visibility of those medicinal products authorised for rare diseases prior to April 2000 when the Orphan legislation was implemented in the EU.

Participants in the meeting agreed that the orphan initiative had provided an important stimulus to the development of medicinal products and there would be an interest in exploring similar initiatives for medical devices.

Detailed proceedings of the meeting will be published by the EMEA in due course.

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/

For further information, please contact:
Martin Harvey Allchurch, EMEA press officer
Tel. (+44-20) 74 18 84 27, E-mail: press@emea.eu.int

ANNEX

List of Participants
Second EMEA Workshop on Orphan Medicinal Products with Patient's Representatives and Learned Societies

held on 4 March 2005

COMP Chairperson:
Prof Josep TORRENT-FARNELL

Learned Societies

Prof H.G.M. Leufkens	Dutch Steering Commitee Orphan Drugs
Ms Irene Roberts	European Hematology Association (EHA)
Dr Konstantin Stoitchkov	European Organisation for Research and Treatment of Cancer (EORTC)
Prof Roger Finch (University of Nottingham)	European Society of Clinical Microbiology and Infectious Diseases
Dr Ségolène Aymé	Orphanet
Dr Bernhard Grewin	Standing Committee of European Doctors
Dr Paolo Casali	European Societies of Medical Oncology
Mr Tom Parkhill	Society of Endocrinology
Dr Bruce Morland	Birmingham Children's Hospital
Mr José Mª Amate Blanco	Agencia de Evaluación de Tecnologías Sanitarias

Patient Organisations:

Mr Albert van der Zeijden	International Alliance of Patient's Organisations
Mr Stephen Jones	Retina Europe
Mrs Dorthe Lysgaard	KMS (Kontaktudvalget for Mindre Sygdoms-og handicapforeninger)
Mr Flavio Minelli	UNIAMO (Federazione Italiana Malattie Rare)
Mr Moisés Abascal Alonso	FEDER (Federación Española de Enfermedades Raras)
Mr Raoul Dammert	
Ms Lise Murphy	Sällsynta diagnoser
Mrs Diane Barnett	Contact a Family (CAF)

COMP Members:

Dr Veijo Saano	Finland
Dr Rembert Elbers	Germany
Dr Harrie Seeverens	The Netherlands
Dr Greg Markey	United Kingdom
Dr David Lyons	CHMP Representative
Mrs Birthe Byskov Holm	European Organisation for Rare Disorders (EURORDIS)
Dr Sigurdur B. Thorsteinsson	Iceland
Mr Alastair Kent	European Genetic Alliances Network (EGAN)

European Commission

Dr Peter Arlett	DG Enterprise

EMEA Secretariat

Dr Agnès Saint Raymond
Prof Spiros Vamvakas
Dr Jordi Llinares Garcia
Ms Melanie Carr
Ms Agnieszka Wilk-Kachlicka
Ms Cinzia N'Diamoi
Ms Frida Rivière
Ms Teresa Martin del toro
Dr Cristina Rebordosa