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

5 April 2001
COMP/108/01

PRESS RELEASE

1st EMEA Workshop for Patients' Organisations on Orphan Medicinal Products

"Understanding How the System Works and Paving the Way Forward"

On 21 March 2001, the EMEA hosted the 1st Workshop for Patients' Organisations on Orphan Medicinal Products. Thirty-four representatives of Patients' Organisations from across the European Union met with members of the Committee for Orphan Medicinal Products (COMP), the European Commission and representatives of the EMEA. The list of participants is provided in Annex.

Dr. Keith Jones (Chairman of the Management Board of the EMEA) and Mr. Thomas Lönngren (Executive Director of the EMEA) welcomed the participants. The orphan initiative is a priority for the EMEA and the importance of working closely together with patients' organisations was emphasised.

Since EU Legislation[1] on orphan medicinal products was implemented nearly one year ago, the first orphan medicinal products have been designated in the European Union. This was felt to be the ideal occasion to invite representatives of patient organisations to voice their expectations and concerns.

The objectives of this workshop were to establish communication links with representatives of patients' organisations, to disseminate information, and to exchange views and expectations on development and access to orphan medicinal products.

The workshop was introduced by Prof. Josep Torrent-Farnell (COMP Chairman). It focused on the presentation of information relating to the orphan designation process, what has been achieved to date, protocol assistance and the marketing authorisation review process. Mrs. Carole Youngs (Vice-President of EURORDIS), Dr. Kerstin Westermark (COMP Member representing Sweden) and Mr. Alastair Kent (COMP Member representing patients' organisations and Director of EAGS) chaired the sessions respectively. The final session, chaired by Dr. Le Courtois (Head of the Pre-Authorisation Unit, EMEA), concluded with a discussion on the building up of a partnership with patients' organisations and the development of a transparency policy on orphan medicinal products.

With a view to increasing transparency, it was agreed that the following proposals will be taken forward by the EMEA for further consideration:

- provision of more rapid and expanded access to information on orphan medicinal products
- expansion of expert involvement in the designation process
- possibility of increased involvement of patients representatives in COMP meetings and in ad-hoc meetings of the COMP

The importance of adequate funding to the success of the orphan initiative was underlined by all participants. Funding from the European Commission supports EMEA activities and permits fee exemptions to be granted for orphan medicinal products.

The EMEA will develop as a platform for communication with all parties involved in orphan drugs. Links with the European Commission's Directorates and national bodies responsible for public health and research initiatives into rare diseases will be reinforced and meetings with interested parties will be organised.

This workshop saw the launch of the COMP's three-tiered action plan to initiate a dialogue with interested parties, including:

- patient organisations
- pharmaceutical industry
- health professionals and academia.

Participants in this workshop expressed interest in the proposal to organise a 2nd meeting within one year.

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

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 for Patients' Organisations on Orphan Medicinal Products

held on 21 March 2001

Chairman of the Management Board of the EMEA
Dr. Keith Jones

Executive Director of the EMEA
Mr. Thomas Lönngren

COMP Chairperson:
Prof. Josep TORRENT-FARNELL

COMP Vice-Chairperson:

Mr. Yann LE CAM	Representative of Patient Organisation

Patient Organisations:

Mr. Tony LEE	European Alliance of Muscular Dystrophy Associations (EAMDA)
Mrs. Alison BRITON	Society for Mucopolysaccharide Diseases
Mrs. Angela RATCLIFLE	Society for Mucopolysaccharide Diseases
Mrs. Ann PHILLIPS	Association for Glycogen Storage Disease (WC)
Mr. Stephen F. JONES	Retina Europe
Ms. Belinda CUPID	Motor Neurone Disease Association
Mrs Jean PHILLUPS-MARTINSSON	International Prader Willi Syndrome Organisation (IPWSO)
Mr. Jorma KOSKINEN	European Aids Treatment Group (EATG)
Mr. Simon COLLINS	European Aids Treatment Group (EATG)
Mr. Francois HOUYEZ	European Aids Treatment Group (EATG)
Ms. Rosa SANCHEZ DE VEGA	Federación Española de Asociaciones de Enfermedades Raras (FEDER)
Ms. Teresa CABALLERO	Federación Española de Asociaciones de Enfermedades Raras (FEDER)
Ms. Teresa BARCO	Federación Española de Asociaciones de Enfermedades Raras (FEDER)
Mr. Francesc VALENZUELA	Federación Española de Asociaciones de Enfermedades Raras (FEDER)
Mr. Raoul DAMMERT	Sällsynta Diagnoser
Dr. Julie SILVESTER	The Cancer Research Campaign
Mrs. Guilaine ARDUIN	European Organisation for Rare Disorders (EURORDIS)
Mr. Anders OLAUSON	European Organisation for Rare Disorders (EURORDIS)
Ms. Carole YOUNGS	European Organisation for Rare Disorders (EURORDIS)
Ms PATRIDGE	European Organisation for Rare Disorders (EURORDIS)
Ms ROQUES DE BORDA	European Organisation for Rare Disorders (EURORDIS)
Mr. WATTERS	European Organisation for Rare Disorders (EURORDIS)
Mr. Françoise SALAMA	European Organisation for Rare Disorders (EURORDIS)
Mrs. Michel FORST	European Organisation for Rare Disorders (EURORDIS)
Mrs. Hawa DRAME	European Organisation for Rare Disorders (EURORDIS)
Ms Lesley GREENE	Children Living with Inherited Metabolic Disorders (CLIMB)
Mr. Torben GRØNNEBÆK	DARD
Mrs. Isabelle DERAM	Alliance Maladies Rares
Mr. Bernard JACQUES	Alliance Maladies Rares
Mr. Emmanuel TRENADO	Alliance Maladies Rares
Mrs. Christel NOURISSIER	Alliance Maladies Rares
Dr. Cordula HARTER	Bundesarbeitsgemeinschaft Hilfe für Behinderte e.V. (BAGH)
Mr. Christop NACHTIGALLER	Bundesarbeitsgemeinschaft Hilfe für Behinderte e.V. (BAGH)
Dr. Stephanie WEINSEICH	VSOP and Dutch Association Neuromuscular Disease

COMP Members:

Dr. André LHOIR	België/Belgique/Belgien
Dr. Heidrun BOSCH-TRABERG	Danmark
Dr. Rembert ELBERS	Deutschland
Prof. George STATHOPOULOS	Ελλάδα
Dr. José OLALLA MARAÑÓN	España
Dr. Brendan BUCKLEY	Ireland
Dr. Domenica TARUSCIO	Italia
Dr. H.J.J. SEEVERENS	Nederland
Prof.Dr. Hans Georg EICHLER	Österreich
Dr. José Manuel Gião TOSCANO RICO	Portugal
Dr. Kalle HOPPU	Suomi/Finland
Dr. Kerstin WESTERMARK	Sverige
Dr. Rashmi SHAH	United Kingdom
Mr. Abascal ALONSO	Representative of Patient Organisation
Mr. Alastair KENT	Representative of Patient Organisation
Dr. David Lyons	EMEA Representative

European Commission

Ms. Emer COOKE	DG Enterprise

EMEA Secretariat
Dr. Patrick LE COURTOIS
Dr. Spiros VAMVAKAS
Ms. Melanie CARR
Dr. Driss BERDAI
Dr. Rui SANTOS IVO
Dr. Francesco PIGNATTI
Mr. Antoine CUVILLIER
Mr Martin HARVEY
Ms. Theresa MC FADDEN
Ms. Monica GOMAR-MENGOD
Ms. Nicola MARTIN

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