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

17 May 2004
EMEA/COMP/178/04

PRESS RELEASE
Committee for Orphan Medicinal Products
May 2004 Meeting

The forty-sixth meeting of the Committee for Orphan Medicinal Products (COMP) took place on 13-14 May 2004. The Committee was delighted to officially welcome ten COMP members from the new Member States. An updated list of COMP membership is provided in Annex 1.

Mr. Philippe Brunet, Head of Unit Pharmaceuticals: Regulatory Framework and Market Authorisations, Enterprise Directorate-General, European Commission presented the outcome of the review of Pharmaceutical Legislation, the draft Paediatric Regulation and more general issues on orphan medicinal products related in particular to the European Commission Communication. Dr. Alain Vanvossel and Dr. Catherine Berens, Research Directorate-General, European Commission presented an update on the Sixth-Framework Programme.

On 26 April 2004 the Committee adopted one positive opinion via written procedure for the following condition:

- Stenosis in synthetic grafts used in haemodialysis

Five positive opinions on the designation of orphan medicinal products were adopted by the Committee during this meeting, for the following conditions:

- Cutaneous T-cell lymphoma
- Gram negative bacterial lung infection in cystic fibrosis
- MART-1 positive malignant melanoma in HLA-A2 positive patients
- Myasthenia gravis
- Osteosarcoma

Of these five opinions, four were adopted on day 56 of the procedure, well before the 90-day deadline for the Committee's opinion following receipt of a valid application.

One oral explanation took place during the meeting. The COMP noted that two applications for orphan medicinal product designation were withdrawn by sponsors.

The European Commission granted four positive decisions on orphan designation[1] since the last COMP meeting on 14 April 2004 (see Annex 2).
The status of orphan designation procedures, to date for 2004, is summarised below:

Applications submitted	*Positive COMP Opinions*	*Applications withdrawn*	*Appeals ongoing*	*Final negative COMP Opinions*	*Designations granted by Commission*
34	21	9	-	-	16

An overview of orphan designation procedures for 2000-2003 is provided in Annex 3.

Further information on designated orphan medicinal products is publicly available in the form of summarised COMP Opinions[2], which the Agency routinely publishes following adoption of the respective decisions on orphan designation by the European Commission.

[1] Details of all orphan designations granted to date by the European Commission are entered in the Community Register of Orphan Medicinal Products (http://pharmacos.eudra.org/F2/register/index.htm)

[2] These documents are available on the EMEA web-site.

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

The next COMP meeting will be held on 15-17 June 2004.

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: martin.harvey@emea.eu.int

Composition of the Committee for Orphan Medicinal Products

Chair: Josep TORRENT i FARNELL
EMEA contact: Agnès SAINT RAYMOND

Members

- Eric ABADIE (EMEA representative)
- Gianmartino BENZI (EMEA representative)
- Heidrun BOSCH-TRABERG (Denmark)
- Birthe BYSKOV HOLM (patient organisation representative)
- Judit EGGENHOFER (Hungary)
- Rembert ELBERS (Germany)
- José Manuel GIÃO TOSCANO RICO (Portugal)
- Joseph GIGLIO(Malta)
- Lars GRAMSTAD (Norway)
- Emmanuel HERON (France)
- Bernd JILMA (Austria)
- Alastair KENT (patient organisation representative)
- Ioannis KKOLOS (Cyprus)
- Kateřina KUBÁČKOVÁ (Czech Republic)
- Magdaléna KUŽELOVÁ (Slovak Republic)
- Yann LE CAM (patient organisation representative), (vice-chairman)
- André LHOIR (Belgium)
- David LYONS (EMEA representative)
- Henri METZ (Luxembourg)
- Martin MOŽINA (Slovenia)
- José Félix OLALLA MARAÑÓN (Spain)
- Kristina PAVLOVSKA (Latvia)
- Veijo SAANO (Finland)
- Harrie SEEVERENS (The Netherlands)
- Rashmi SHAH (United Kingdom)
- George SHORTEN (Ireland)
- George STATHOPOULOS (Greece)
- Domenica TARUSCIO (Italy)
- Sigurdur THORSTEINSSON (Iceland)
- Vallo TILLMANN (Estonia)
- Algirdas UTKUS (Lithuania)
- Kerstin WESTERMARK (Sweden)
- Jolanta WIĘCKOWSKA (Poland)

Medicinal products Designated as Orphan Medicinal Products since the April 2004 COMP Meeting

Active substance	Treprostinil sodium (inhalation use)
Sponsor	LungRx Limited
Orphan Indication	Treatment of pulmonary arterial hypertension and chronic thromboembolic pulmonary hypertension
Opinion receipt date	23/3/2004
Date of Commission Decision	14/4/2004

Active substance	Human monoclonal antibody against CD4
Sponsor	Genmab A/S
Orphan Indication	Treatment of cutaneous T-cell lymphoma
Opinion receipt date	23/3/2004
Date of Commission Decision	14/4/2004

Active substance	Ciclosporin
Sponsor	Allergan Pharmaceuticals Ireland
Orphan Indication	Treatment of atopic keratoconjunctivitis
Opinion receipt date	19/3/2004
Date of Commission Decision	14/4/2004

Active substance	2-Methoxy-5-[(1Z)-2-(3,4,5-trimethoxyphenyl)ethenyl]-phenol
Sponsor	Dr David Chaplin
Orphan Indication	Treatment of anaplastic thyroid cancer
Opinion receipt date	19/3/2004
Date of Commission Decision	14/4/2004

Overview of Procedures for Orphan Medicinal Product Designation for 2000-2003

Year	*Applications submitted*	*Positive COMP Opinions*	*Applications withdrawn*[3]	*Final negative COMP Opinions*	*Designations granted by Commission*
2003	87	54	41	1	55
2002	80	43	30	3	49
2001	83	64	27	1	64
2000	72	26	6	0	14

[3] These figures have been updated to include withdrawals from the validation phase of the designation procedure