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

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PRESS RELEASE
Committee for Orphan Medicinal Products
May 2006 Meeting

The sixty-eight meeting of the Committee for Orphan Medicinal Products (COMP) took place on 15-16 May 2006.

This was the first meeting of the third COMP mandate. The Committee welcomed warmly the following new members: Dr Bożenna Dembowska-Bagińska (Poland), Dr Julia Dunne (CHMP representative), Dr Pauline Evers (patient's organization representative), Dr Aušra Matulevičienė (Lithuania), Dr Miranda Siouti (Greece), Dr Evija Strode (Latvia), Prof. Josep Torrent-Farnell (Spain), Dr Bettie Voordouw (The Netherlands). The new Chair and Vice-Chair will be elected during the meeting in June.

COMP Opinions

The Committee adopted 7 positive opinions on orphan medicinal product designation during this meeting:

- Diphenylcyclopropenone, from Orfagen, for treatment of **alopecia universalis** (review time: day 87)
- Diphenylcyclopropenone, from Orfagen, for treatment of **alopecia totalis** (review time: day 87)
- Human monoclonal antibody against *Pseudomonas aeruginosa* serotype O11, from MDS Pharma Services GB Limited, for treatment of **pneumonia caused by serotype O11 *Pseudomonas aeruginosa*** (review time: day 59)
- Mecasermin rinfabate, from Insmed Europe Ltd., for treatment of **primary insulin-like growth factor-1 deficiency due to molecular or genetic defects** (review time: day 30)
- Mecasermin rinfabate, from Insmed Europe Ltd., for treatment of **patients with growth hormone (GH) gene deletion who have developed neutralizing antibodies to GH** (review time: day 30)
- Pazopanib hydrochloride, from GlaxoSmithKline Research & Development Limited, for treatment of **renal cell carcinoma** (review time: day 59)
- Siplizumab, from MedImmune Oncology, Inc., for treatment of **T-cell and NK-cell neoplasms** (review time: day 59)

Two oral explanations took place during the meeting. The COMP noted that one application for orphan medicinal product designation was withdrawn by the sponsor during the validation phase of the procedure.

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Overview of orphan designation procedures

The European Commission granted nine positive decisions on orphan designation[1] since the last COMP meeting on 4-5 April 2006.

The status of orphan designation procedures, to date for 2006, is summarised below:

Applications submitted	*Positive COMP Opinions*	*Applications withdrawn*	*Appeals ongoing*	*Final negative COMP Opinions*	*Designations granted by Commission*
29	35	7	-	-	24

An overview of orphan designation procedures for 2000-2005 is provided in Annex 1.

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.

Useful information to sponsors

It has been agreed that information on conditions that do not fulfill the criteria for Orphan Designation will be published as of next month. This may cover the prevalence of conditions considered by the COMP to be above 5/10,000 or conditions not considered to meet the seriousness criterion. The information given will not be product related. The aim is to give information on the experience accumulated by the Committee and increase the likelihood of success for applications.

Applications for Marketing Authorisation for Orphan Medicinal Products

Details of those designated orphan medicinal products that have been the subject of a centralised application for marketing authorisation since the last COMP meeting are provided in Annex 2.

Date of next COMP meeting

The next COMP meeting will be held on 14-15 June 2006.

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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[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.

Orphan Medicinal Product Designations received since the April 2006 COMP Meeting

Active substance	4-[^{131}I] iodo-L-phenylalanine
Sponsor	Dr Andreas Kluge
Orphan Indication	Treatment of glioma
Opinion receipt date	23/03/2006
Date of Commission Decision	11/04/2006

Active substance	Adeno associated viral vector containing the human calpain 3 gene
Sponsor	Généthon
Orphan Indication	Treatment of calpainopathy
Opinion receipt date	23/03/2006
Date of Commission Decision	6/04/2006

Active substance	Ciclosporin
Sponsor	Novagali Pharma
Orphan Indication	Treatment of vernal keratoconjunctivitis
Opinion receipt date	23/03/2006
Date of Commission Decision	6/04/2006

Active substance	Glutathione
Sponsor	Mukoviszidose e.V.
Orphan Indication	Treatment of cystic fibrosis
Opinion receipt date	23/03/2006
Date of Commission Decision	11/04/2006

Active substance	Human heterologous liver cells (for infusion)
Sponsor	Cytonet GmbH & Co. KG
Orphan Indication	Treatment of acute liver failure
Opinion receipt date	23/03/2006
Date of Commission Decision	11/04/2006

Active substance	Parathyroid hormone (1-34) transglutaminase fusion protein fibrin matrix complex
Sponsor	Kuros Biosurgery International AG
Orphan Indication	Treatment of solitary bone cysts
Opinion receipt date	23/03/2006
Date of Commission Decision	11/04/2006

Active substance	Sorafenib tosylate
Sponsor	Bayer HealthCare AG
Orphan Indication	Treatment of hepatocellular carcinoma
Opinion receipt date	23/03/2006
Date of Commission Decision	11/04/2006

Active substance	Temsirolimus
Sponsor	Wyeth Europa Limited
Orphan Indication	Treatment of renal cell carcinoma
Opinion receipt date	23/03/2006
Date of Commission Decision	6/04/2006

Active substance	Tobramycin (liposomal)
Sponsor	EUCRO GmbH & Co. KG
Orphan Indication	Treatment of *Pseudomonas aeruginosa* lung infection in cystic fibrosis
Opinion receipt date	23/03/2006
Date of Commission Decision	11/04/2006

Overview of Procedures for Orphan Medicinal Product Designation for 2000-2005

Year	*Applications submitted*	*Positive COMP Opinions*	*Applications withdrawn*	*Final negative COMP Opinions*	*Designations granted by Commission*
2005	118	88	30	0	88
2004	108	75	22	4	72
2003	87	54	41	1	55
2002	80	43	30	3	49
2001	83	64	27	1	64
2000	72	26	6	0	14

Overview of Designated Orphan Medicinal Products that have been the subject of a Centralised Application for Marketing Authorisation

- update since the last COMP meeting on 4-5 April 2006 -

Active substance	***Sponsor/applicant***	***EU Designation Number & Date of Orphan Designation***	***Designated Orphan Indication***
5-Aminolevulinic acid hydrochloride (Gliolan)	Medac Gesellschaft für klinische Spezialpräparate mbH	EU/3/02/121 13/11/2002	Intra-operative photodynamic diagnosis of residual glioma