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

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PRESS RELEASE
Committee for Orphan Medicinal Products
December 2004 Meeting

The fifty-second meeting of the Committee for Orphan Medicinal Products (COMP) took place on 7-8 December 2004.

The Committee welcomed two new members, Dr. Greg Markey will replace Dr. Rashmi Shah as the UK COMP member; Dr. Patrick Salmon, the new Irish COMP member, replaces Prof. George Shorten who resigned from the Committee in July 2004. The Committee thanked Dr. Shah and Prof. Shorten for their contribution and collaboration.

COMP Opinions

The Committee adopted 5 positive opinions on orphan medicinal product designation during this meeting and one positive opinion via written procedure following the November meeting:

- Recombinant human α-Mannosidase, from HemeBiotech A/S, for treatment of **α-mannosidosis** (review time: 52 days).
- 17-allylamino-17-demethoxygeldanamycin, from Wainwright Associates Limited, for treatment of **chronic myeloid leukaemia** (review time: 52 days).
- Acetylcysteine, from Zambon Group SpA, for treatment of **idiopathic pulmonary fibrosis** (review time: 52 days).
- L-Asparaginase, from Medac Gesellschaft für klinische Spezialpräparate mbH, for treatment of **acute lymphoblastic leukaemia** (review time: 90 days).
- Recombinant human bile salt-stimulated lipase, from Arexis AB, for treatment of **cystic fibrosis** (review time: 90 days).
- N-(methyl-diazacyclohexyl-methylbenzamide)-azaphenyl-aminothiopyrrole, from AB Science, for treatment of **malignant gastro intestinal stromal tumours** (review time: 93 days).

Two oral explanations took place during the meeting. The COMP noted that three applications for orphan medicinal product designation were withdrawn by sponsors, 2 during the validation phase and 1 following evaluation.

Overview of orphan designation procedures

The European Commission granted six positive decisions on orphan designation[1] since the last COMP meeting on 10-11 November 2004 (see Annex 1).

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Applications submitted	*Positive COMP Opinions*	*Applications withdrawn*	*Appeals ongoing*	*Final negative COMP Opinions*	*Designations granted by Commission*
98	75	20	2	1	63

An overview of orphan designation procedures for 2000-2003 is provided in Annex 2.

[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)

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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.

Revised COMP Rules of Procedure

During this meeting, the Committee agreed upon a revision to the Rules of Procedure of the Committee. The Rules have been revised in line with those of other EMEA Committees in accordance with Regulation (EC) No 726/2004. The revised Rules of Procedure (EMEA/8212/00 Rev 2) are available on the EMEA web-site.

Date of next COMP meeting

The next COMP meeting will be held on 13 January 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/

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

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

Active substance	Alpha-1 antitrypsin (inhalation use)
Sponsor	BCG (Europe) Ltd
Orphan Indication	Treatment of cystic fibrosis
Opinion receipt date	15/10/2004
Date of Commission Decision	16/11/2004

Active substance	Alpha-1 antitrypsin (inhalation use)
Sponsor	BCG (Europe) Ltd
Orphan Indication	Treatment of emphysema secondary to congenital alpha-1 antitrypsin deficiency
Opinion receipt date	15/10/2004
Date of Commission Decision	16/11/2004

Active substance	Aplidine
Sponsor	Pharma Mar SA Sociedad Unipersonal
Orphan Indication	Treatment of multiple myeloma
Opinion receipt date	15/10/2004
Date of Commission Decision	16/11/2004

Active substance	N-(methyl-diazacyclohexyl-methylbenzamide)-azaphenyl-aminothiopyrrole
Sponsor	AB Science
Orphan Indication	Treatment of mastocytosis.
Opinion receipt date	15/10/2004
Date of Commission Decision	16/11/2004

Active substance	Pirfenidone
Sponsor	Uppsala Medical Information System AB
Orphan Indication	Treatment of idiopathic pulmonary fibrosis
Opinion receipt date	15/10/2004
Date of Commission Decision	16/11/2004

Active substance	Valproic acid, sodium
Sponsor	G2M Cancer Drugs AG
Orphan Indication	Treatment of Familial Adenomatous Polyposis
Opinion receipt date	22/10/2004
Date of Commission Decision	30/11/2004

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

Year	*Applications submitted*	*Positive COMP Opinions*	*Applications withdrawn*	*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