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

8 October 2004
EMEA/COMP/101411/2004

PRESS RELEASE
Committee for Orphan Medicinal Products
October 2004 Meeting

The fiftieth meeting of the Committee for Orphan Medicinal Products (COMP) took place on 6-7 October 2004.

COMP Opinions

The Committee adopted 6 positive opinions on orphan medicinal product designation for:

- Aplidine, from Pharma Mar SA Sociedad Unipersonal, for treatment of **multiple myeloma** (review time: 88 days).
- Alpha-1 antitrypsin (inhalation use), from BCG (Europe) Ltd, for treatment of **cystic fibrosis** (review time: 53 days).
- Alpha-1 antitrypsin (inhalation use), from BCG (Europe) Ltd, for treatment of **emphysema secondary to congenital alpha-1-antitrypsin deficiency** (review time: 53 days).
- N-(methyl-diazacyclohexyl-methylbenzamide)-azaphenyl-aminothiopyrrole, from AB Science, for treatment of **mastocytosis** (review time: 53 days).
- Pirfenidone, from Uppsala Medical Information System AB, for treatment of **idiopathic pulmonary fibrosis** (review time: 53 days).
- Valproic acid, from G2M Cancer Drugs AG, for treatment of **familial adenomatous polyposis** (review time: 53 days).

One oral explanation took place during the meeting, resulting in a withdrawal. The COMP noted that, since the last COMP meeting on 8-9 September 2004, a further three applications had been withdrawn by sponsors.

Two negative opinions on orphan medicinal product designation were adopted via written procedure on 21 September 2004. The sponsor has notified its intent to appeal the negative Opinions.

Overview of orphan designation procedures

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Applications submitted	*Positive COMP Opinions*	*Applications withdrawn*	*Appeals ongoing*	*Final negative COMP Opinions*	*Designations granted by Commission*
80	61	15	-	-	44

An overview of orphan designation procedures for 2000-2003 is provided in Annex 1.

Further information on designated orphan medicinal products is publicly available in the form of summarised COMP Opinions[1], which the Agency routinely publishes following adoption of the respective decisions on orphan designation by the European Commission.

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

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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 10-11 November 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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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

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 8-9 Spetember 2004 -

Active substance	***Sponsor/applicant***	***EU Designation Number & Date of Orphan Designation***	***Designated Orphan Indication***
2-chloro-9-[2-deoxy-2-fluoro-ß-D-arabinofuranosyl]adenine (Clofarabine-Bioenvision Limited)	Bioenvision Limited	EU/3/01/082 05/02/2002	Treatment of acute lymphoblastic leukaemia
		EU/3/03/141 08/05/2003	Treatment of acute myeloid leukaemia
Azacitidine (Vidaza)	Pharmion Limited	EU/3/01/084 06/02/2002	Treatment of myelodysplastic syndromes