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

18 January 2005
EMEA/COMP/19824/2005

PRESS RELEASE
Committee for Orphan Medicinal Products
January 2005 Meeting

The fifty-third meeting of the Committee for Orphan Medicinal Products (COMP) took place on 13 January 2005.

COMP Opinions

The Committee adopted 3 positive opinions on orphan medicinal product designation during this meeting:

- Fluocinolone acetonide (prolonged-release intravitreal implant), from Bausch & Lomb (UK) Ltd, for treatment of **non-infectious uveitis affecting the posterior segment of the eye** (review time: 67 days).
- Dimethyl sulfoxide, from AOP Orphan Pharmaceuticals AG, for treatment of **severe closed traumatic brain injury** (review time: 67 days).
- Titanium dioxide and bisoctrizole, from Orfagen, for treatment of **UV-A and visible light-induced photosensitivity disorders (chronic actinic dermatitis, cutaneous porphyrias, actinic prurigo and solar urticaria)** (review time: 67 days).

Two oral explanations took place during the meeting. The COMP noted that three applications for orphan medicinal product designation were withdrawn by sponsors, including two at the end of last year.

Overview of orphan designation procedures

The European Commission granted nine positive decisions on orphan designation[1] since the last COMP meeting on 7-8 December 2004 (see Annex 1).

The status of orphan designation procedures, to date for 2005, is summarised below:

Applications submitted	*Positive COMP Opinions*	*Applications withdrawn*	*Appeals ongoing*	*Final negative COMP Opinions*	*Designations granted by Commission*
-	3	1	1	-	-

An overview of orphan designation procedures for 2000-2004 is provided in Annex 2.

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.

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 3.

[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.

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Date of next COMP meeting

The next COMP meeting will be held on 2-3 February 2005.

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

Active substance	N-(methyl-diazacyclohexyl-methylbenzamide)-azaphenyl-aminothiopyrrole
Sponsor	AB Science
Orphan Indication	Treatment of malignant gastro intestinal stromal tumours
Opinion receipt date	19/11/2004
Date of Commission Decision	21/12/2004

Active substance	17-allylamino-17-demethoxygeldanamycin
Sponsor	Wainwright Associates Limited
Orphan Indication	Treatment of Multiple Myeloma
Opinion receipt date	19/11/2004
Date of Commission Decision	21/12/2004

Active substance	Recombinant histidine-tagged idiotype immunoglobulin Fab fragment of clonal B-cell receptors
Sponsor	CellGenix Technologie Transfer GmbH
Orphan Indication	Treatment of multiple myeloma
Opinion receipt date	19/11/2004
Date of Commission Decision	21/12/2004

Active substance	Recombinant histidine-tagged idiotype immunoglobulin Fab fragment of clonal B-cell receptors
Sponsor	CellGenix Technologie Transfer GmbH
Orphan Indication	Treatment of mantle cell lymphoma
Opinion receipt date	19/11/2004
Date of Commission Decision	21/12/2004

Active substance	Recombinant histidine-tagged idiotype immunoglobulin Fab fragment of clonal B-cell receptors
Sponsor	CellGenix Technologie Transfer GmbH
Orphan Indication	Treatment of follicular lymphoma
Opinion receipt date	19/11/2004
Date of Commission Decision	23/12/2004

Active substance	Val-Leu-Gln-Glu-Leu-Asn-Val-Thr-Val (Pr1 nanopeptide, sequence 169-177, of proteinase 3)
Sponsor	Accelsiors CRO & Consultancy Services GmbH
Orphan Indication	Treatment of myelodysplastic syndromes
Opinion receipt date	19/11/2004
Date of Commission Decision	21/12/2004

Active substance	Val-Leu-Gln-Glu-Leu-Asn-Val-Thr-Val (Pr1 nanopeptide, sequence 169-177, of proteinase 3)
Sponsor	Accelsiors CRO & Consultancy Services GmbH
Orphan Indication	Treatment of chronic myeloid leukemia
Opinion receipt date	19/11/2004
Date of Commission Decision	21/12/2004

Active substance	Val-Leu-Gln-Glu-Leu-Asn-Val-Thr-Val (Pr1 nanopeptide, sequence 169-177, of proteinase 3)
Sponsor	Accelsiors CRO & Consultancy Services GmbH
Orphan Indication	Treatment of acute myeloid leukaemia
Opinion receipt date	19/11/2004
Date of Commission Decision	21/12/2004

Active substance	Sabarubicin
Sponsor	Menarini Ricerche S.p.A.
Orphan Indication	Treatment of small cell lung cancer
Opinion receipt date	19/11/2004
Date of Commission Decision	21/12/2004

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*
2004	108	75	22	1	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 7-8 December 2004 -

Active substance	***Sponsor/applicant***	***EU Designation Number & Date of Orphan Designation***	***Designated Orphan Indication***
N-acetylgalactosamine-4-sulfatase (Galsulfase-Biomarin)	BioMarin Europe Ltd	EU/3/01/025 14/02/2001	Treatment of Mucopolysaccharidosis, type VI (Maroteaux-Lamy Syndrome).
Recombinant human acid α-glucosidase (Myozyme)	Genzyme Europe BV	EU/3/01/018 14/02/2001	Treatment of Glycogen Storage Disease type II (Pompe's disease).
Sildenafil citrate (Revatio)	Pfizer Limited	EU/3/03/178 12/12/2003	Treatment of pulmonary arterial hypertension and chronic thromboembolic pulmonary hypertension.