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

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PRESS RELEASE
Committee for Orphan Medicinal Products
April 2005 Meeting

The fifty-sixth meeting of the Committee for Orphan Medicinal Products (COMP) took place on 6-7 April 2005.

The Committee welcomed Dr. Brad Glasscock, Senior Reviewing Pharmacist, from the Office for Orphan Product Development (OOPD) at the FDA. Dr. Glasscock updated the Committee on the activities of the OOPD.

COMP Opinions

The Committee adopted 9 positive opinions on orphan medicinal product designation during this meeting:

- (3-[5-(2-fluoro-phenyl)-[1,2,4]oxadiazole-3-yl]-benzoic acid, from the Matthews Consultancy Ltd, for treatment of **cystic fibrosis** (review time: 89 days).
- (3-[5-(2-fluoro-phenyl)-[1,2,4]oxadiazole-3-yl]-benzoic acid, from the Matthews Consultancy Ltd, for treatment of **Duchenne muscular dystrophy** (review time: 89 days).
- (E)-(1S,4S,10S,21R)-7-[(Z)-ethylidene]-4,21-diisopropyl-2-oxa-12,13-dithia-5,8,20,23-tetraazabicyclo[8.7.6]tricos-16-ene-3,6,9,19,22-pentone, from the Matthews Consultancy Ltd, for treatment of **cutaneous T-cell lymphoma** (review time: 54 days).
- Acadesine, from Advanced In Vitro Cell Technologies, for treatment of **B-cell chronic lymphocytic leukemia** (review time: 54 days).
- Bimosiamose, from Revotar Biopharmaceuticals AG, for treatment of **acute lung injury** (review time: 54 days).
- Interferon gamma, from Mondobiotech Laboratories Anstalt, for treatment of **idiopathic pulmonary fibrosis** (review time: 54 days).
- Miltefosine, from Obwaller Forschungs- und Entwicklungs GmbH, for treatment of **acathamoeba keratitis** (review time: 54 days).
- Recombinant megakaryopoiesis-stimulating protein, from Amgen Europe BV, for treatment of **idiopathic thrombocytopenic purpura** (review time: 54 days).
- Sodium butyrate (rectal use), from Promefarm srl, for prevention of **radiation proctitis** (review time: 54 days).

Three oral explanations took place during the meeting. The COMP noted that three applications for orphan medicinal product designation were withdrawn by sponsors.

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

Overview of orphan designation procedures

The European Commission granted nine positive decisions on orphan designation[1] since the last COMP meeting on 2-3 March 2005 (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*
28	25	8	-	-	14

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.

Date of next COMP meeting

The next COMP meeting will be held on 11-12 May 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/

For further information, please contact:
Martin Harvey Allchurch, EMEA press officer
Tel. (+44-20) 74 18 84 27, E-mail: press@emea.eu.int

[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.

Medicinal products Designated as Orphan Medicinal Products since the March 2005 COMP Meeting

Active substance	Cholest-4-en-3-one, oxime
Sponsor	Trophos SA
Orphan Indication	Treatment of 5q spinal muscular atrophy
Opinion receipt date	18/02/2005
Date of Commission Decision	10/03/2005

Active substance	Ciclosporin (inhalation use)
Sponsor	Chiron Corporation Ltd
Orphan Indication	Prevention of graft rejection after lung transplantation
Opinion receipt date	18/02/2005
Date of Commission Decision	10/03/2005

Active substance	Ciclosporin (inhalation use)
Sponsor	Chiron Corporation Ltd
Orphan Indication	Treatment of graft rejection after lung transplantation
Opinion receipt date	18/02/2005
Date of Commission Decision	10/03/2005

Active substance	(Z)-N-[2-(Diethylamino)ethyl]-5-[(5-fluoro-2-oxo-1,2-dihydro-3Hindol-3-ylidene)methyl]-2,4-dimethyl-1H-pyrrole-3-carboxamide (S)-2-hydroxysuccinate
Sponsor	Pfizer Limited
Orphan Indication	Treatment of malignant gastrointestinal stromal tumours
Opinion receipt date	18/02/2005
Date of Commission Decision	10/03/2005

Active substance	(Z)-N-[2-(Diethylamino)ethyl]-5-[(5-fluoro-2-oxo-1,2-dihydro-3Hindol-3-ylidene)methyl]-2,4-dimethyl-1H-pyrrole-3-carboxamide (S)-2-hydroxysuccinate
Sponsor	Pfizer Limited
Orphan Indication	Treatment of renal cell carcinoma
Opinion receipt date	18/02/2005
Date of Commission Decision	10/03/2005

Active substance	Dimethyl sulfoxide
Sponsor	AOP Orphan Pharmaceuticals AG
Orphan Indication	Treatment of severe closed traumatic brain injury
Opinion receipt date	03/02/2005
Date of Commission Decision	03/03/2005

Active substance	Fluocinolone acetonide (prolonged-release intravitreal implant)
Sponsor	Bausch & Lomb (UK) Ltd
Orphan Indication	Treatment of noninfectious uveitis affecting the posterior segment of the eye
Opinion receipt date	03/02/2005
Date of Commission Decision	07/03/2005

Active substance	Titanium dioxide and bisoctrizole
Sponsor	Orfagen
Orphan Indication	Treatment of UV-A and visible light-induced photosensitivity disorders (chronic actinic dermatitis, cutaneous porphyrias, actinic prurigo and solar urticaria)
Opinion receipt date	03/02/2005
Date of Commission Decision	07/03/2005

Active substance	Tipifarnib
Sponsor	Janssen-Cilag International NV
Orphan Indication	Treatment of acute myeloid leukaemia
Opinion receipt date	10/03/2005
Date of Commission Decision	18/02/2005

Overview of Procedures for Orphan Medicinal Product Designation for 2000-2004

Year	*Applications submitted*	*Positive COMP Opinions*	*Applications withdrawn*	*Final negative COMP Opinions*	*Designations granted by Commission*
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 2-3 March 2005 -

Active substance	***Sponsor/applicant***	***EU Designation Number & Date of Orphan Designation***	***Designated Orphan Indication***
Recombinant human monoclonal antibody to hsp90 (Mycograb)	NeuTec Pharma plc	EU/3/01/073 05/12/2001	Treatment of invasive fungal infections
Rufinamide (Inovelon)	Eisai Limited	EU/3/04/240 20/10/2004	Treatment of Lennox-Gastaut syndrome