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

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PRESS RELEASE
Committee for Orphan Medicinal Products
April 2007 Meeting

The seventy-eighth meeting of the Committee for Orphan Medicinal Products (COMP) took place on 11-12 April 2007.

COMP Opinions for Orphan Medicinal Product Designation

The Committee adopted 6 positive opinions on orphan medicinal product designation during this meeting:

- 5(S)-(2'-hydroxy ethoxy)-20(S)-camptothecin, from ClinTec International Ltd, **for treatment of osteosarcoma** (review time: day 39)
- Cisplatin (liposomal), from Regulon AE, **for treatment of pancreatic cancer** (review time: day 88)
- Everolimus, from Novartis Europharm Limited, **for treatment of renal cell carcinoma** (review time: day 39)
- R-1-[2,3-dihydro-2-oxo-1-pivaloylmethyl-5-(2-pyridyl)-1H-1,4-benzodiazepin-3-yl]-3-(3-methylaminophenyl)urea, from Trio Medicines Ltd, **for treatment of gastric carcinoid** (review time: day 39)
- Recombinant fusion protein consisting of human coagulation factor IX attached to the Fc domain of human IgG1, from Biovitrum AB, **for treatment of haemophilia B (congenital factor IX deficiency)** (review time: day 39)
- Talactoferrinum alfa, from Agennix Limited, **for treatment of renal cell carcinoma** (review time: day 39)

Withdrawals of Orphan Medicinal Product Applications

The COMP noted that no application for orphan medicinal product designation was withdrawn.

Overview of orphan designation procedures

The European Commission granted eight positive decisions on orphan designation[1] since the last COMP meeting on 7-8 March 2007 (see Annex 1).

[1] Details of all orphan designations granted to date by the European Commission are entered in the Community Register of Orphan Medicinal Products (http://ec.europa.eu/enterprise/pharmaceuticals/index_en.htm)

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The status of orphan designation procedures, to date for 2007, is summarised below:

Applications submitted	*Positive COMP Opinions*	*Applications withdrawn*	*Appeals ongoing*	*Final negative COMP Opinions*	*Designations granted by Commission*
32	24	6	-	-	20

An overview of orphan designation procedures for 2000-2006 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.

COMP Opinions on article 5 (12) of Regulation (EC) No 141/2000 of the European Parliament and of the Council

The Committee adopted 2 opinions on the maintenance of the Orphan Medicinal Product designation criteria prior to the market authorisation during this meeting:

- 3-(4'aminoisoindoline-1'-one)-1-piperidine-2,6-dione, from Celgene Europe limited, **for treatment of multiple myeloma**

- Bosentan, from Actelion Registration Ltd., **for treatment of systemic sclerosis**

Following the receipt of the grounds for appeal and its evaluation the negative opinion adopted in November 2006 by the Committee was maintained.

Date of next COMP meeting

The next COMP meeting will be held on 30-31 May and 1 June 2007.

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.europa.eu.

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

Orphan Medicinal Product Designations received since the March 2007 COMP Meeting

Active substance	Elafin
Sponsor	Proteo Biotech AG
Orphan Indication	Treatment of pulmonary hypertension and chronic thromboembolic pulmonary hypertension
Opinion receipt date	1 March 2007
Date of Commission Decision	20 March 2007

Active substance	Enzastaurin hydrochloride
Sponsor	Eli Lilly Nederland B.V.
Orphan Indication	Treatment of diffuse large B cell lymphoma
Opinion receipt date	22 February 2007
Date of Commission Decision	20 March 2007

Active substance	Eptacog alfa (activated)
Sponsor	Novo Nordisk A/S
Orphan Indication	Treatment of post-neonatal intracerebral haemorrhage
Opinion receipt date	22 February 2007
Date of Commission Decision	20 March 2007

Active substance	Hydrocortisone (modified release tablet)
Sponsor	Phoqus Pharmaceuticals Ltd
Orphan Indication	Treatment of adrenal insufficiency
Opinion receipt date	22 February 2007
Date of Commission Decision	20 March 2007

Active substance	Idebenone
Sponsor	Santhera Pharmaceuticals (Deutchland) AG
Orphan Indication	Treatment of Duchenne muscular dystrophy
Opinion receipt date	22 February 2007
Date of Commission Decision	20 March 2007

Active substance	Recombinant adeno-associated viral vector containing human alpha-1 antitrypsin gene
Sponsor	The Matthews Consultancy Ltd
Orphan Indication	Treatment of alpha-1 antitrypsin deficiency
Opinion receipt date	22 February 2007
Date of Commission Decision	20 March 2007

Active substance	Recombinant human monoclonal antibody to human IL-1beta of the IgG1/K class
Sponsor	Novartis Europharm Limited
Orphan Indication	Treatment of cryopirin-associated periodic syndromes (Familial Ciold Urticaria Syndrome (FCUS), Muckle-Wells Syndrome (MWS), and Neonatal Onset Multisystem Inflammatory Disease (NOMID), also known as Chronic Infantile Neurological Cutaneous
Opinion receipt date	22 February 2007
Date of Commission Decision	20 March 2007

Active substance	Zanolinumab
Sponsor	Serono Europe Ltd.
Orphan Indication	Treatment of peripheral T-cell lymphoma (nodal, other extranodal and leukaemic/disseminated)
Opinion receipt date	22 February 2007
Date of Commission Decision	20 March 2007

Overview of Procedures for Orphan Medicinal Product Designation for 2000-2006

Year	*Applications submitted*	*Positive COMP Opinions*	*Applications withdrawn*	*Final negative COMP Opinions*	*Designations granted by Commission*
2006	104	81	20	2	80
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 7-8 March 2007-

Active substance	***Sponsor/applicant***	***EU Designation Number & Date of Orphan Designation***	***Designated Orphan Indication***
Ambrisentan (Volibris)	Glaxo Group Limited-UK	EU/3/05/273 11/04/2005	Treatment of pulmonary arterial hypertension and chronic thromboembolic pulmonary hyperetension