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

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PRESS RELEASE
Committee for Orphan Medicinal Products
June 2007 Meeting

The eightieth meeting of the Committee for Orphan Medicinal Products (COMP) took place on 26-27 June 2007.

COMP Opinions for Orphan Medicinal Product Designation

The Committee adopted 10 positive opinions on orphan medicinal product designation during this meeting:

- Arsenic trioxide, Cephalon Europe, **for treatment of acute myeloid leukaemia** (review time: day 62)
- Ciprofloxacin (inhalation use), from Bayer Healthcare AG, **for treatment of cystic fibrosis** (review time: day 90)
- Dihydroartemisinin, piperaquine, from Sigma Tau Industrie Farmaceutiche Riunite S.p.A, **for treatment of malaria** (review time: day 90)
- Human plasminogen, from Kedrion S.p.A, **for treatment of ligneous conjunctivitis** (review time: day 62)
- Eltrombopag olamine, GlaxoSmithKline Research & Development Limited, **for treatment of idiopathic thrombocytopenic purpura** (review time: day 62)
- L-threo-3,4-dihydroxyphenylserine, The Weinberg Group LLC, **for treatment of orthostatic hypotension in patients with multiple system atrophy** (review time: day 62)
- L-threo-3,4-dihydroxyphenylserine, The Weinberg Group LLC, **for treatment of orthostatic hypotension in patients with pure autonomic failure** (review time: day 62)
- Panobinostat lactate, from Novartis Europharm Limited, **for treatment of cutaneous T-Cell Lymphoma** (review time: day 62)
- Pyridoxalated hemoglobin polyoxyethylene, Curacyte AG, **for treatment of cardiogenic shock** (review time: day 62)
- Recombinant human soluble Fc-gamma receptor II b, SuppreMol GmbH, **for treatment of idiopathic thrombocytopenic purpura** (review time: day 62)

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Overview of orphan designation procedures

The European Commission granted 4 positive decisions on orphan designation[1] since the last COMP meeting on 30-31 May 2007 (see Annex 1).

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Applications submitted	*Positive COMP Opinions*	*Applications withdrawn*	*Appeals ongoing*	*Final negative COMP Opinions*	*Designations granted by Commission*
61	39	9	-	-	28

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:

- 5-aminolevulonic acid hydrochloride, from medac Gesellschaft für klinische Spezialpräparate mbH, **for treatment of glioma***
- Nelarabine, from GlaxoSmithKline Research & Development Limited, **for treatment of acute lymphoblastic leukaemia**

Prior to this meeting, the Committee adopted one opinion on the maintenance of the Orphan Medicinal Product designation criteria prior to the market authorisation via written procedure on 11 June 2007:

- Mecasermin, from Tercica Europe Limited, **for treatment of primary insulin-like growth factor-1 deficiency due to molecular or genetic disorders**

Date of next COMP meeting

The next COMP meeting will be held on 24-25 July 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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[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)

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

* Amended indication

Orphan Medicinal Product Designations received since the May 2007 COMP Meeting

Active substance	5(S)-(2'-hydroxy ethoxy)-20(S)-camptothecin
Sponsor	ClinTec International Ltd
Orphan Indication	Treatment of osteosarcoma
Opinion receipt date	4 May 2007
Date of Commission Decision	8 June 2007

Active substance	Cisplatin (liposomal)
Sponsor	Regulon AE
Orphan Indication	Treatment of pancreatic cancer
Opinion receipt date	4 May 2007
Date of Commission Decision	8 June 2007

Active substance	Recombinant fusion protein consisting of human coagulation factor IX attached to the Fc domain of human IgG1
Sponsor	Biovitrum AB
Orphan Indication	Treatment of haemophilia B (congenital factor IX deficiency)
Opinion receipt date	4 May 2007
Date of Commission Decision	8 June 2007

Active substance	R-1-[2,3-dihydro-2-oxo-1-pivaloylmethyl-5-(2-pyridyl)-1 H-1,4-benzodiazepin-3-yl]-3-(3-methylaminophenyl)urea
Sponsor	Trio Medicines Ltd
Orphan Indication	Treatment of gastric carcinoid
Opinion receipt date	4 May 2007
Date of Commission Decision	14 June 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 30-31May 2007-

Active substance	***Sponsor/applicant***	***EU Designation Number & Date of Orphan Designation***	***Designated Orphan Indication***
Sorafenib tosylate (Nexavar)	Bayer HealthCare AG	EU/3/06/364 11/05/2006	Treatment of hepatocellular carcinoma