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

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PRESS RELEASE
Committee for Orphan Medicinal Products
March 2007 Meeting

The seventy-seventh meeting of the Committee for Orphan Medicinal Products (COMP) took place on 7-8 March 2007.

COMP Opinions

The Committee adopted 4 positive opinions on orphan medicinal product designation during this meeting:

- Antisense oligonucleotide (TATCCGGAGGGCTCGCCATGCTGCT), from Gene Signal SAS, **for prevention of corneal graft rejection** (review time: day 88)

- Autologous CD34+ cells transfected with lentiviral vector containing the human arylsulfatase A cDNA, from Fondazione Telethon, **for treatment of metachromatic leukodystrophy** (review time: day 53)

- Nilotinib hydrochloride monohydrate, from Novartis Europharm Limited, **for treatment of gastrointestinal stromal tumours** (review time: day 88)

- Pralatrexate, from Oxford Regulatory Solutions Ltd, **for treatment of peripheral T-cell lymphoma** (nodal, other extranodal and leukaemic/disseminated)(review time: day 53)

Two oral explanations on orphan medicinal product designation took place during the meeting.

Prior to this meeting, the Committee adopted one positive opinion via written procedure on 12 February 2007:

- Eptacog alfa (activated), from Novo Nordisk A/S, **for treatment of intracerebral haemorrhage** (review time: day 95)

Withdrawals of Orphan Medicinal Product Applications

The COMP noted that two applications for orphan medicinal product designation were withdrawn at evaluation.

Overview of orphan designation procedures

The European Commission granted seven positive decisions on orphan designation[1] since the last COMP meeting on 6-7 February 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Applications submitted	*Positive COMP Opinions*	*Applications withdrawn*	*Appeals ongoing*	*Final negative COMP Opinions*	*Designations granted by Commission*
14	18	6	-	-	12

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.

Date of next COMP meeting

The next COMP meeting will be held on 11-12 April 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 February 2007 COMP Meeting

Active substance	Artesunate
Sponsor	ACE Pharmaceuticals BV
Orphan Indication	Treatment of Malaria
Opinion receipt date	24 January 2007
Date of Commission Decision	20 February 2007

Active substance	Autologous dendritic cells pulsed with autologous tumour cell lysate
Sponsor	Dorian Regulatory Affairs BV
Orphan Indication	Treatment of glioma
Opinion receipt date	24 January 2007
Date of Commission Decision	15 February 2007

Active substance	Ex-vivo cultured adult human mesenchymal stem cells
Sponsor	Quintiles UK Limited
Orphan Indication	Treatment of Graft-versus-Host disease
Opinion receipt date	24 January 2007
Date of Commission Decision	20 February 2007

Active substance	Fenretinide
Sponsor	Cancer Research UK
Orphan Indication	Treatment of soft tissue sarcoma
Opinion receipt date	21 December 2006
Date of Commission Decision	30 January 2007

Active substance	HLA class I/II binding tumour associated peptides (ADF-APO-CCN-GUC-K67-MET-MMP-MUC-RGS)
Sponsor	Immatics Biotechnologies GmbH
Orphan Indication	Treatment of renal cell carcinoma
Opinion receipt date	24 January 2007
Date of Commission Decision	15 February 2007

Active substance	Idebenone
Sponsor	Santhera Pharmaceuticals (Deutchland) GmbH
Orphan Indication	Treatment of Leber's hereditary optic neuropaty
Opinion receipt date	24 January 2007
Date of Commission Decision	15 February 2007

Active substance	Recombinant human C1-inhibitor
Sponsor	Pharming Group N.V.
Orphan Indication	Prevention of delayed graft function after solid organ transplantation
Opinion receipt date	24 January 2007
Date of Commission Decision	20 February 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 6-7 February 2007-

Active substance	***Sponsor/applicant***	***EU Designation Number & Date of Orphan Designation***	***Designated Orphan Indication***
Thalidomide (Thalidomide Pharmion 50mg Hard Capsules)	Pharmion Ltd	EU/3/01/067 20/11/2001	Treatment of multiple myeloma
2-chloro-9-[2-deoxy-2-fluoro-ß-D-arabinofuranosyl]adenine (Evoltra)	Bioenvision limited	EU/3/03/141 08/05/2003	Treatment of acute myeloid leukaemia