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

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PRESS RELEASE
Committee for Orphan Medicinal Products
June 2006 Meeting

The sixty-ninth meeting of the Committee for Orphan Medicinal Products (COMP) took place on 14-15 June 2006.

During this meeting, the members of the Committee elected the new Chairperson and the Vice-Chairperson for a term of three years. Dr Kerstin Westermark (Sweden) was elected unanimously as Chairperson, and Mrs. Birthe Byskov Holm (Patient Representative nominated by the European Commission) was elected unanimously as Vice-Chairperson. The Committee and Executive Director of the European Medicines Agency congratulated the new Chairperson and Vice-Chairperson.

COMP Opinions

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

- 4-[^{123}I] iodo-L-phenylalanine, from Dr. Andreas Kluge, for **diagnosis of glioma** (review time: day 87)
- 2-(4-(diethylamino) phenyl)-6-methyl-2H-benzo[d][1,2,3] triazol-5-amine, from Vastox Plc, for **treatment of Duchenne muscular dystrophy** (review time: day 58)
- Amikacin sulfate (liposomal), from Morgan Lewis & Bockius, for **treatment *Pseudomonas aeruginosa* lung infection in cystic fibrosis** (review time: day 58)
- Becatecarin, from Helsinn Birex Pharmaceuticals Ltd for **treatment of cancers of the biliary tree** (review time: day 58)
- Human telomerase reverse transcriptase peptide (611-626), from Pharmexa A/S, **for treatment of pancreatic cancer** (review time: day 58)
- Lestaurtinib, from Cephalon UK Ltd, for **treatment of acute myeloid leukaemia** (review time: day 58)

Two oral explanations took place during the meeting.

Withdrawals of Orphan Medicinal Product Applications

The COMP noted that two applications for orphan medicinal product designation were withdrawn by sponsors during the evaluation phase of the procedure.

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Overview of orphan designation procedures

The European Commission granted eight positive decisions on orphan designation[1] since the last COMP meeting on 15-16 May 2006 (see Annex 1).

The status of orphan designation procedures, to date for 2006, is summarised below:

Applications submitted	*Positive COMP Opinions*	*Applications withdrawn*	*Appeals ongoing*	*Final negative COMP Opinions*	*Designations granted by Commission*
42	41	9	-	-	32

An overview of orphan designation procedures for 2000-2005 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 11-13 July 2006.

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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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.

Orphan Medicinal Product Designations received since the May 2006 COMP Meeting

Active substance	1-deoxygalactonojirimycin hydrochloride
Sponsor	Amicus Therapeutics UK Limited
Orphan Indication	Treatment of Fabry disease
Opinion receipt date	24 April 2006
Date of Commission Decision	22 May 2006

Active substance	Bilayer engineered skin composed of keratinocytes from the patient (autologous) and fibroblasts from a donor (allogeneic) embedded in a plasma matrix
Sponsor	Cellerix S.L.
Orphan Indication	Treatment of epidermolysis bullosa
Opinion receipt date	24 April 2006
Date of Commission Decision	22 May 2006

Active substance	Heparin sodium
Sponsor	Ockham Biotech Limited
Orphan Indication	Treatment of cystic fibrosis
Opinion receipt date	24 April 2006
Date of Commission Decision	22 May 2006

Active substance	Hydrocortisone (modified release tablet)
Sponsor	DuoCort AB
Orphan Indication	Treatment of Adrenal Insufficiency
Opinion receipt date	24 April 2006
Date of Commission Decision	22 May 2006

Active substance	Mecasermin
Sponsor	Tercica Europe Limited
Orphan Indication	Treatment of primary insulin-like growth factor-1 deficiency due to molecular or genetic defects
Opinion receipt date	24 April 2006
Date of Commission Decision	22 May 2006

Active substance	Methoxsalen
Sponsor	Johnson & Johnson Medical Ltd
Orphan Indication	Treatment of Graft-versus-Host disease
Opinion receipt date	24 April 2006
Date of Commission Decision	22 May 2006

Active substance	Nilotinib
Sponsor	Novartis Europharm Limited
Orphan Indication	Treatment of chronic myeloid leukaemia
Opinion receipt date	24 April 2006
Date of Commission Decision	22 May 2006

Active substance	Recombinant P-selectin glycoprotein immunoglobulin
Sponsor	RJM Consultancy Ltd
Orphan Indication	Prevention of post transplantation graft dysfunction
Opinion receipt date	24 April 2006
Date of Commission Decision	22 May 2006

Overview of Procedures for Orphan Medicinal Product Designation for 2000-2005

Year	*Applications submitted*	*Positive COMP Opinions*	*Applications withdrawn*	*Final negative COMP Opinions*	*Designations granted by Commission*
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 15-16 May 2006 -

Active substance	***Sponsor/applicant***	***EU Designation Number & Date of Orphan Designation***	***Designated Orphan Indication***
Nelarabine (Atriance)	Glaxo Group Limited	EU/3/05/293 16/06/2005	Treatment of acute lymphoblastic leukaemia
Bosentan (Tracleer)	Actelion Registration Limited	EU/3/03/139 17/03/2003	Treatment of systemic sclerosis (scleroderma)