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

12 January 2006
EMEA/COMP/11156/2006

PRESS RELEASE
Committee for Orphan Medicinal Products
January 2006 Meeting

The sixty-fourth meeting of the Committee for Orphan Medicinal Products (COMP) took place on 10-11 January 2006.

The Committee welcomed Mr. Jean George from Altzheimer Europe who attended the meeting as Patients' Organisation representative at the EMEA Management Board.

COMP Opinions

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

- 26 base single stranded phosphodiester DNA oligonucleotide, from Antisoma Research Ltd, for treatment of **pancreatic cancer** (review time: 59 days).
- 26 base single stranded phosphodiester DNA oligonucleotide, from Antisoma Research Ltd, for treatment of **renal cell carcinoma** (review time: 59 days).
- 2'-O-methyl-phosphorothioate oligonucleotide, from Prosensa B.V., for treatment of **Duchenne muscular dystrophy** (review time: 59 days).
- 4-amino-5-oxo-4 (pyridinium-1-ylmethyl) proline, from Prodimed S.A., for treatment of **renal cell carcinoma** (review time: 59 days).
- Alpha 1 - proteinase inhibitor, from Octapharma (IP) Limited, for treatment of **emphysema secondary to congenital alpha 1 - antitrypsin deficiency** (review time: 59 days).
- Apomorphine hydrochloride (inhalation use), from Vectura Group plc, for treatment of **off-periods in Parkinson's disease not responding to oral treatment** (review time: 87 days).
- Miglustat, from Actelion Registration Ltd, for treatment of **Niemann-Pick disease, type C** (review time: 59 days).
- *Oxalobacter formigenes* strain HC-1, from OxThera AB, for treatment of **primary hyperoxaluria** (review time: 59 days).
- Zosuquidar trihydrochloride, from Uppsala Medical Information System AB, for treatment of **acute myeloid leukaemia** (review time: 59 days).

Two oral explanations took place during the meeting. The COMP noted that one application for orphan medicinal product designation was withdrawn by the sponsor during the evaluation phase of the procedure.

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Overview of orphan designation procedures

The European Commission granted twelve positive decisions on orphan designation[1] since the last COMP meeting on 7 December 2005 (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*
1	9	1	-	-	-

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 a one-day meeting to be held on 7 February 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 December 2005 COMP Meeting

Active substance	Chimeric monoclonal antibody to shiga-toxin 1 and 2
Sponsor	Albany Regulatory Consulting Ltd
Orphan Indication	Treatment of shiga-toxin producing bacterial infection.
Opinion receipt date	04/08/2005
Date of Commission Decision	26/08/2005

Active substance	1,2-bis(methylsulphonyl)-1-(2-chloroethyl)-2-[(methylamino)carbonyl]hydrazine
Sponsor	Vion (UK) Limited
Orphan Indication	Treatment of acute myeloid leukaemia
Opinion receipt date	14/11/2005
Date of Commission Decision	14/12/2005

Active substance	Brostallicin
Sponsor	Nerviano Medical Science Srl
Orphan Indication	Treatment of soft tissue sarcoma
Opinion receipt date	23/11/2005
Date of Commission Decision	23/12/2005

Active substance	Dasatinib
Sponsor	Bristol-Myers Squibb Pharma EEIG
Orphan Indication	Treatment of acute lymphoblastic leukaemia
Opinion receipt date	23/11/2005
Date of Commission Decision	23/12/2005

Active substance	Dasatinib
Sponsor	Bristol-Myers Squibb Pharma EEIG
Orphan Indication	Treatment of chronic myeloid leukaemia
Opinion receipt date	23/11/2005
Date of Commission Decision	23/12/2005

Active substance	Denufosol tetrasodium
Sponsor	Quintiles UK Limited
Orphan Indication	Treatment of cystic fibrosis
Opinion receipt date	23/11/2005
Date of Commission Decision	23/12/2005

Active substance	Enzastaurin hydrochloride
Sponsor	Eli Lilly Nederland B.V.
Orphan Indication	Treatment of glioma
Opinion receipt date	23/11/2005
Date of Commission Decision	23/12/2005

Active substance	Eptacog alfa (activated)
Sponsor	DKA Consult ApS
Orphan Indication	Treatment of diffuse alveolar haemorrhage
Opinion receipt date	14/11/2005
Date of Commission Decision	14/12/2005

Active substance	Heparin sodium (inhalation use)
Sponsor	Vectura Group plc
Orphan Indication	Treatment of cystic fibrosis
Opinion receipt date	23/11/2005
Date of Commission Decision	23/12/2005

Active substance	Human immunoglobulin G1 constant region - human ectodysplasin-A1 receptor-binding domain fusion protein
Sponsor	Apoxis SA
Orphan Indication	Treatment of X-linked hypohidrotic ectodermal dysplasia (Christ-Siemens-Touraine Syndrome)
Opinion receipt date	14/11/2005
Date of Commission Decision	14/12/2005

Active substance	Human *Staphylococcus aureus* polyclonal immunoglobulin and human *Staphylococcus epidermidis* polyclonal immunoglobulin
Sponsor	Omnicare Clinical Research
Orphan Indication	Prevention of late onset sepsis in premature infants of less or equal than 32 weeks gestational age
Opinion receipt date	14/11/2005
Date of Commission Decision	14/12/2005

Active substance	Imatinib mesilate
Sponsor	Novartis Europharm Limited
Orphan Indication	Treatment of myelodysplastic/myeloproliferative diseases
Opinion receipt date	23/11/2005
Date of Commission Decision	23/12/2005

Active substance	Imexon
Sponsor	ICON Clinical Research (U.K.) Limited
Orphan Indication	Treatment of ovarian cancer
Opinion receipt date	23/11/2005
Date of Commission Decision	23/12/2005

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

* Please note that these are the revised and confirmed final figures for 2005.

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 December 2005 -

Active substance	***Sponsor/applicant***	***EU Designation Number & Date of Orphan Designation***	***Designated Orphan Indication***
Gemtuzumab ozogamicin (Mylotarg)	Wyeth Europa Limited	EU/3/00/005 18/10/2000	Treatment of patients with acute myeloid leukaemia
Iduronate-2-sulfatase (Zipralase)	TKT UK Ltd	EU/3/01/078 11/12/2001	Treatment of mucopolysaccharidosis, type II (Hunter syndrome)
Imatinib mesilate (Glivec)	Novartis Europharm Limited	EU/3/05/305 26/08/2005	Treatment of dermatofibrosarcoma protuberans
Imatinib mesilate (Glivec)	Novartis Europharm Limited	EU/3/05/304 26/08/2005	Treatment of acute lymphoblastic leukaemia
Imatinib mesilate (Glivec)	Novartis Europharm Limited	EU/3/05/340 23/12/2005	Treatment of myelodysplastic/myeloproliferative diseases