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

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PRESS RELEASE
Committee for Orphan Medicinal Products
October 2005 Meeting

The sixty-first meeting of the Committee for Orphan Medicinal Products (COMP) took place on 19 October 2005.

COMP Opinions

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

- Human *Staphylococcus aureus* polyclonal immunoglobulin and human *Staphylococcus epidermidis* polyclonal immunoglobulin, from Omnicare Clinical Research, for prevention of **late onset sepsis in premature infants of less or equal than 32 weeks gestational age** (review time: 89 days).
- 1,2-bis(methylsulphonyl)-1-(2-chloroethyl)-2-[(methylamino)carbonyl]hydrazine, from Vion (UK) Limited, for treatment of **acute myeloid leukaemia** (review time: 71 days).
- Human immunoglobulin G1 constant region - human ectodysplasin-A1 receptor-binding domain fusion protein, from Apoxis SA, for treatment of **X-linked hypohidrotic ectodermal dysplasia (Christ-Siemens-Touraine Syndrome)** (review time: 71 days).
- Eptacog alfa (activated), from DKA Consult ApS, for treatment of **diffuse alveolar haemorrhage** (review time: 71 days).

One oral explanation took place during the meeting. The COMP noted that sponsors withdrew five applications for orphan medicinal product designation during the validation phase of the procedure.

Overview of orphan designation procedures

The European Commission granted nine positive decisions on orphan designation[1] since the last COMP meeting on 8-9 September 2005 (see Annex 1).

The status of orphan designation procedures, to date for 2005, is summarised below:

Applications submitted	*Positive COMP Opinions*	*Applications withdrawn*	*Appeals ongoing*	*Final negative COMP Opinions*	*Designations granted by Commission*
91	75	22	-	-	61

An overview of orphan designation procedures for 2000-2004 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.

[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.

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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 9-10 November 2005.

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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Medicinal products Designated as Orphan Medicinal Products since the September 2005 COMP Meeting

Active substance	(1R,2R,4S)-4-{(2R)-2-[(3S,6R,7E,9R,10R,12R,14S,15E,17E,19E,21S,23S,26R,27R,34aS)-9,27-dihydroxy-10,21-dimethoxy-6,8,12,14,20,26-hexamethyl-1,5,11,28,29-pentaoxo-1,4,5,6,9,10,11,12,13,14,21,22,23,24,25,26,27,28,29,31,32,33,34,34a-tetra-cosahydro-3H-23,27-epoxypyrido[2,1-c][1,4]oxazacyclohentriacontin-3-yl]propyl}-2-methoxy-cyclohexyldimethyl-phosphinate
Sponsor	Orphix Consulting GmbH
Orphan Indication	Treatment of soft tissue sarcoma
Opinion receipt date	2/08/2005
Date of Commission Decision	26/08/2005

Active substance	Imatinib mesilate
Sponsor	Novartis Europharm Limited
Orphan Indication	Treatment of dermatofibrosarcoma protuberans
Opinion receipt date	2/08/2005
Date of Commission Decision	26/08/2005

Active substance	Imatinib mesilate
Sponsor	Novartis Europharm Limited
Orphan Indication	Treatment of acute lymphoblastic leukaemia
Opinion receipt date	2/08/2005
Date of Commission Decision	26/08/2005

Active substance	Imatinib mesilate
Sponsor	Novartis Europharm Limited
Orphan Indication	Treatment of mastocytosis
Opinion receipt date	2/08/2005
Date of Commission Decision	26/08/2005

Active substance	Mecasermin
Sponsor	MDS Pharma Services GB Limited
Orphan Indication	Treatment of primary growth hormone insensitivity syndrome
Opinion receipt date	2/08/2005
Date of Commission Decision	26/08/2005

Active substance	Sapropterin
Sponsor	Dr Gunter Schaub
Orphan Indication	Treatment of hyperphenylalaninemia
Opinion receipt date	2/08/2005
Date of Commission Decision	26/08/2005

Active substance	Sodium valproate
Sponsor	The Jennifer Trust for Spinal Muscular Atrophy
Orphan Indication	Treatment of 5q spinal muscular atrophy
Opinion receipt date	2/08/2005
Date of Commission Decision	26/08/2005

Active substance	Treprostinil diethanolamine (oral use)
Sponsor	United Therapeutics Europe Ltd
Orphan Indication	Treatment of pulmonary arterial hypertension and chronic thromboembolic pulmonary hypertension
Opinion receipt date	2/08/2005
Date of Commission Decision	26/08/2005

Active substance	Troxacitabine
Sponsor	GMG BioBusiness Ltd
Orphan Indication	Treatment of acute myeloid leukaemia
Opinion receipt date	2/08/2005
Date of Commission Decision	26/08/2005

Overview of Procedures for Orphan Medicinal Product Designation for 2000-2004

Year	*Applications submitted*	*Positive COMP Opinions*	*Applications withdrawn*	*Final negative COMP Opinions*	*Designations granted by Commission*
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 8-9 September 2005 -

Active substance	***Sponsor/applicant***	***EU Designation Number & Date of Orphan Designation***	***Designated Orphan Indication***
Sorafenib tosylate (Nexavar)	Bayer Healthcare AG	EU/3/04/207 29/07/2004	Treatment of renal cell carcinoma
3-(4'aminoisoindoline-1'-one)-1-piperidine-2,6-dione (Revlimid)	Celgene Europe Limited	EU/3/04/192 8/03/2004	Treatment of myelodysplastic syndromes
(Z)-N-[2-(Diethylamino)ethyl]-5-[(5-fluoro-2-oxo-1,2-dihydro-3H-indol-3-ylidene)methyl]-2,4-dimethyl-1H-pyrrole-3-carboxamide (S)-2-hydroxysyccinate (Sutent)	Pfizer Limited	EU/3/05/267 10/03/2005	Treatment of malignant gastrointestinal stromal tumours
(Z)-N-[2-(Diethylamino)ethyl]-5-[(5-fluoro-2-oxo-1,2-dihydro-3H-indol-3-ylidene)methyl]-2,4-dimethyl-1H-pyrrole-3-carboxamide (S)-2-hydroxysyccinate (Sutent)	Pfizer Limited	EU/3/05/268 10/03/2005	Treatment of renal cell carcinoma