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

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PRESS RELEASE
Committee for Orphan Medicinal Products
November 2005 Meeting

The sixty-second meeting of the Committee for Orphan Medicinal Products (COMP) took place on 9 – 10 November 2005.

COMP Opinions

The Committee adopted 8 positive opinions on orphan medicinal product designation during this meeting:

- Brostallicin, from Nerviano Medical Science Srl, for treatment of **soft tissue sarcoma** (review time: 60 days).
- Dasatinib, from Bristol-Myers Squibb Pharma EEIG, for treatment of **acute lymphoblastic leukaemia** (review time: 60 days).
- Dasatinib, from Bristol-Myers Squibb Pharma EEIG, for treatment of **chronic myeloid leukaemia** (review time: 60 days).
- Denufosol tetrasodium, from Quintiles Limited, for treatment of **cystic fibrosis** (review time: 60 days).
- Enzastaurin, from Eli Lilly Nederland B.V., for treatment of **glioma** (review time: 60 days).
- Heparin sodium (inhalation use), from Vectura Group plc, for treatment of **cystic fibrosis** (review time: 88 days).
- Imatinib mesilate, from Novartis Europharm Limited, for treatment of **myelodysplastic/myeloproliferative diseases** (review time: 88 days).
- Imexon, from ICON Clinical Research (UK) Ltd, for treatment of **ovarian cancer** (review time: 88 days).

Three oral explanations took place during the meeting. The COMP noted that sponsors withdrew three applications for orphan medicinal product designation during the evaluation phase of the procedure.

Overview of orphan designation procedures

The European Commission granted ten positive decisions on orphan designation[1] since the last COMP meeting on 19 October 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Applications submitted	*Positive COMP Opinions*	*Applications withdrawn*	*Appeals ongoing*	*Final negative COMP Opinions*	*Designations granted by Commission*
102	83	25	-	-	71

[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)

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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.

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

Medicinal products Designated as Orphan Medicinal Products since the October 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 primary malignant bone tumours
Opinion receipt date	22/09/2005
Date of Commission Decision	28/10/2005

Active substance	(*E*)-(1*S*,4*S*,10*S*,21*R*)-7-[(*Z*)-ethylidene]-4,21-diisopropyl-2-oxa-12,13-dithia-5,8,20,23-tetraazabicyclo [8.7.6]tricos-16-ene-3,6,9,19,22-pentone
Sponsor	The Matthews Consultancy Ltd.
Orphan Indication	Treatment of peripheral T-cell lymphoma (nodal, other extranodal and leukaemic/disseminated)
Opinion receipt date	22/09/2005
Date of Commission Decision	28/10/2005

Active substance	Alfimeprase
Sponsor	QuadraMed Limited
Orphan Indication	Treatment of acute peripheral arterial occlusion
Opinion receipt date	22/09/2005
Date of Commission Decision	28/10/2005

Active substance	Bacterial lipase
Sponsor	Nordmark Arzneimittel GmbH u. Co. KG
Orphan Indication	Treatment of malabsorption due to exocrine pancreatic enzyme insufficiency
Opinion receipt date	22/09/2005
Date of Commission Decision	07/11/2005

Active substance	Levamisol hydrochloride
Sponsor	ACE Pharmaceuticals BV
Orphan Indication	Treatment of nephrotic syndrome
Opinion receipt date	22/09/2005
Date of Commission Decision	28/10/2005

Active substance	Mannitolum
Sponsor	Stricent AB
Orphan Indication	Treatment of cystic fibrosis
Opinion receipt date	22/09/2005
Date of Commission Decision	07/11/2005

Active substance	Oligonucleotide phosphorothioate (TAAACGTTATAACGTTATGACGTCAT), sodium salt
Sponsor	Oligovax
Orphan Indication	Treatment of glioma
Opinion receipt date	22/09/2005
Date of Commission Decision	28/10/2005

Active substance	Peptide 144 TGF-beta1-inhibitor (TSLDASIIWAMMQN)
Sponsor	Digna Biotech S.L.
Orphan Indication	Treatment of localised scleroderma
Opinion receipt date	22/09/2005
Date of Commission Decision	28/10/2005

Active substance	Recombinant microbial lipase
Sponsor	Solvay Pharmaceuticals GmbH
Orphan Indication	Treatment of malabsorption due to exocrine pancreatic enzyme insufficiency
Opinion receipt date	22/09/2005
Date of Commission Decision	28/10/2005

Active substance	Tilarginine acetate
Sponsor	Dr Ulrich Granzer
Orphan Indication	Treatment of cardiogenic shock
Opinion receipt date	22/09/2005
Date of Commission Decision	03/11/2005

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 19 October 2005 -

Active substance	***Sponsor/applicant***	***EU Designation Number & Date of Orphan Designation***	***Designated Orphan Indication***
Adenovirus-mediated *Herpes simplex* virus – thymidine kinase (HSV-tk) gene (Cerepro)	Ark Therapeutics Ltd	EU/3/01/083 6/02/2002	Treatment of high-grade glioma with subsequent use of ganciclovir sodium
Hydroxyurea (Siklos)	OTL Pharma	EU/3/03/154 9/07/2003	Treatment of sickle cell syndrome