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

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COMMITTEE FOR ORPHAN MEDICINAL PRODUCTS
JANUARY 2008 PLENARY MEETING
MONTHLY REPORT

The Committee for Orphan Medicinal Products (COMP) held its eighty-sixth plenary meeting on 9-10 January 2008.

ORPHAN MEDICINAL PRODUCT DESIGNATION

The COMP adopted four positive opinions recommending the following medicines for designation as orphan medicinal products to the European Commission:

- **Heterologous human adult liver derived stem cells,** from Prof Etienne Sokal, for treatment of ornithinine transcarbamylase deficiency. EMEA review began on 9 November 2007 with an active review time of 63 days.
- **Lumiliximab,** from Biogen Idec Limited, for treatment of chronic lymphocytic leukaemia. EMEA review began on 15 October 2007 with an active review time of 88 days.
- **Recombinant human monoclonal antibody to human IL-1beta of the IgG1/K class,** from Novartis Europharm Limited, for treatment of systemic-onset juvenile idiopathic arthritis. EMEA review began on 9 November 2007 with an active review time of 63 days.
- **Tretazicar,** from Morvus Technology Limited, for treatment of visceral leishmaniasis. EMEA review began on 15 October 2007 with an active review time of 88 days.

Public summaries of opinion will be available on the EMEA website which the Agency updates following adoption of the respective decisions on orphan designation by the European Commission.

OTHER INFORMATION ON THE ORPHAN MEDICINAL PRODUCT DESIGNATION

Lists of questions

The COMP adopted four lists of questions on initial applications. These applications will be discussed again at the next COMP plenary meeting prior to adoption of the opinion.

Oral hearings

Three oral hearings took place.

Withdrawals of applications for orphan medicinal product designation

The COMP noted that two applications for orphan medicinal product designation were withdrawn.

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Detailed information on the orphan designation procedure

An overview of orphan designation procedures since 2000 is provided in **Annex 1**.

The list of medicinal products for which decisions on orphan designation[1] have been given by the European Commission since the last COMP plenary meeting is provided in **Annex 2**.

Applications for marketing authorisation for orphan medicinal products

Details of those designated orphan medicinal products that have been subject of a new community marketing authorisation application through the centralised procedure since the last COMP plenary meeting are provided in **Annex 3**.

Details on the opinions for marketing authorisation for orphan medicinal products adopted by the Committee for Medicinal Products for Human Use (CHMP) can be found in the CHMP Monthly Report on the EMEA website.

UPCOMING MEETINGS FOLLOWING THE JANUARY 2008 COMP PLENARY MEETING

- The eighty-seventh meeting of the COMP will be held on 5-6 February 2008.

ORGANISATIONAL MATTERS

The main topics addressed during the January 2008 COMP meeting related to:

- Discussion on the increasing visibility of authorised orphan products on the EMEA website.
- Discussion with industry representatives on the orphan legislation
- Presentation by Dr K. Kubáčková on targeted therapies in cancer treatment
- Three Protocol Assistance letters were adopted.

NOTE: This Monthly Report and other documents may be found on the internet at the following location: http://www.emea.europa.eu

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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://ec.europa.eu/enterprise/pharmaceuticals/index_en.htm)

OVERVIEW FOR ORPHAN MEDICINAL PRODUCT DESIGNATION PROCEDURE SINCE 2000

Year	Applications submitted	Positive COMP Opinions	Applications withdrawn	Final negative COMP Opinions	Designations granted by Commission
2008	-	4	2	-	-
2007	125	97	19	1	94
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

MEDICINAL PRODUCTS GRANTED A COMMUNITY DESIGNATION AS ORPHAN MEDICINAL PRODUCT SINCE THE DECEMBER COMP PLENARY REPORT BY THE EUROPEAN COMMISSION

Active substance	3-methoxy-pregnenolone
Sponsor	MAPREG SAS
Orphan Indication	Treatment of spinal cord injury
COMP Opinion date	10/10/2007
Orphan Designation date	04/12/2007

Active substance	17-(allylamino)-17-demethoxygeldanamycin, hydroquinone, hydrochloride
Sponsor	MedImmune Oncology, Inc.
Orphan Indication	Treatment of malignant gastrointestinal stromal tumours
COMP Opinion date	10/10/2007
Orphan Designation date	29/11/2007

Active substance	(1R,2R)-Octanoic acid[2-(2',3'-dihydro-benzo[1,4] dioxin-6'-yl)-2-hydroxy-1-pyrrolidin-1-ylmethyl-ethyl]-amide-L-tartaric acid salt
Sponsor	Genzyme Europe BV
Orphan Indication	Treatment of Gaucher Disease
COMP Opinion date	10/10/2007
Orphan Designation date	04/12/2007

Active substance	(S)-2-nitro-6-(4-trifluoromethoxy)benzyloxy)-6,7-dihydro-5H-imidazo[2,1-b] [1,3] oxazine
Sponsor	Dr Ulrich Granzer
Orphan Indication	Treatment of tuberculosis
COMP Opinion date	10/10/2007
Orphan Designation date	29/11/2007

Active substance	Artesunate
Sponsor	Sigma-tau Pharma UK
Orphan Indication	Treatment of malaria
COMP Opinion date	10/10/2007
Orphan Designation date	06/12/2007

Active substance	Chimeric-anti-interleukin 6 monoclonal antibody
Sponsor	Centocor, B.V.

Orphan Indication	Treatment of Castleman's disease
COMP Opinion date	10/10/2007
Orphan Designation date	30/11/2007

Active substance	Doxorubicin hydrochloride (drug eluting beads)
Sponsor	Cellmed AG
Orphan Indication	Treatment of glioma
COMP Opinion date	10/10/2007
Orphan Designation date	29/11/2007

Active substance	Human papilloma virus type 16 E6/E7synthetic long peptides
Sponsor	ISA Pharmaceuticals BV
Orphan Indication	Treatment of epithelial neoplasia of the vulva positive for human papilloma virus
COMP Opinion date	08/11/2007
Orphan Designation date	20/12/2007

Active substance	H-Arg-Leu-Phe-Phe-Tyr-Arg-Lys-Ser-Val-OH, acetate salt & H-Tyr-Leu-Phe-Phe-Tyr-Arg-Lys-Ser-Val-OH, acetate salt
Sponsor	Vaxon Biotech
Orphan Indication	Treatment of TERT positive non-small cell lung cancer in HLA-A2 positive patients
COMP Opinion date	08/11/2007
Orphan Designation date	18/12/2007

Active substance	Lenalidomide
Sponsor	Celgene Europe Limited
Orphan Indication	Treatment of chronic lymphocytic leukaemia
COMP Opinion date	12/09/2007
Orphan Designation date	19/11/2007

Active substance	Maribavir
Sponsor	ViroPharma Limited
Orphan Indication	Prevention of cytomegalovirus (CMV) disease in patients with impaired cell mediated immunity deemed at risk
COMP Opinion date	23/11/2007
Orphan Designation date	18/12/2007

Active substance	Olaparib
Sponsor	AstraZeneca AB
Orphan Indication	Treatment of ovarian cancer
COMP Opinion date	10/10/2007
Orphan Designation date	06/12/2007

Active substance	Picoplatin
Sponsor	Kendle International Ltd
Orphan Indication	Treatment of small cell lung cancer
COMP Opinion date	10/10/2007
Orphan Designation date	06/12/2007

Active substance	Recombinant human hepatitis C monoclonal antibody against C4 region of E1
Sponsor	GENimmune, N.V.
Orphan Indication	Prevention of recurrent hepatitis C virus induced liver disease in liver transplant recipients
COMP Opinion date	10/10/2007
Orphan Designation date	06/12/2007

Active substance	Recombinant human histone H1.3 and recombinant human N-bis-met-histone H1.3
Sponsor	SymbioTec GmbH
Orphan Indication	Treatment of acute myeloid leukaemia
COMP Opinion date	08/11/2007
Orphan Designation date	20/12/2007

Active substance	Recombinant human rod derived cone viability factor
Sponsor	Fovea Pharmaceuticals SA
Orphan Indication	Treatment of retinitis pigmentosa
COMP Opinion date	10/10/2007
Orphan Designation date	29/11/2007

Active substance	Terguride
Sponsor	Ergonex Licensing and Regulatory Services AG
Orphan Indication	Treatment of pulmonary arterial hypertension and chronic thromboembolic pulmonary hypertension
COMP Opinion date	10/10/2007
Orphan Designation date	29/11/2007

DESIGNATED ORPHAN MEDICINAL PRODUCTS THAT HAVE BEEN SUBJECT OF A NEW COMMUNITY MARKETING AUTHORISATION APPLICATION UNDER THE CENTRALISED PROCEDURE SINCE THE DECEMBER 2007 COMP MONTHLY REPORT

Active substance	Invented name	Sponsor/applicant	EU Designation Number	Designated Orphan Indication
Advexin	Adenoviral vector containing human p53 gene	Gendux Molecular Limited	EU/3/06/404	Treatment of Li Fraumeni Syndrome
Ixiaro	Purified inactivated Japanese encephalitis SA 14-4-2 virus vaccine	Intercell AG	EU/3/05/348	Prevention of Japanese encephalitis