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

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COMMITTEE FOR ORPHAN MEDICINAL PRODUCTS
NOVEMBER 2007 PLENARY MEETING
MONTHLY REPORT

The Committee for Orphan Medicinal Products (COMP) held its eighty-fourth plenary meeting on 7-8 November 2007.

ORPHAN MEDICINAL PRODUCT DESIGNATION

The COMP adopted 7 positive opinions recommending the following medicines for designation as orphan medicinal products to the European Commission:

- **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,** from Vaxon Biotech for treatment of TERT positive non-small cell lung cancer in HLA-A2 positive patients. EMEA review began on 13 August 2007 with an active review time of 88 days.
- **Human papilloma virus type 16 E6/E7 synthetic long peptides,** from ISA Pharmaceuticals BV, for treatment of epithelial neoplasia of the vulva positive for human papilloma virus. EMEA review began on 13 August 2007 with an active review time of 88 days.
- **Maribavir,** form ViroPharma Limited, for prevention of cytomegalovirus (CMV) disease in patients with impaired cell medical immunity deemed at risk. EMEA review began on 13 August 2007 with an active review time of 88 days.
- **Methyl 4,6-diamino-2-[1-(2-fluorobenzyl)-1H-pyrazolo[3,4-b]pyridine-3-yl]-5-pyrimidinyl(methyl)carbamate,** from Bayer HealthCare AG, for treatment of pulmonary arterial hypertension including treatment of chronic thromboembolic pulmonary hypertension. EMEA review began on 10 September 2007 with an active review time of 60 days.
- **N-[4-(3-amino-1H-indazol-4 yl)phenyl]-N'-(2-fluoro-5-methylphenyl) urea,** from Abbott Laboratories, for treatment of hepatocellular carcinoma. EMEA review began on 10 September 2007 with an active review time of 60 days.
- **Recombinant human histone H1.3 and recombinant human N-bis-met-histone H1.3,** from SymbioTec GmbH, for treatment of acute myeloid leukaemia. EMEA review began on 10 September 2007 with an active review time of 60 days.
- **Tegafur, gimeracil, oteracil potassium,** from Sanofi Aventis, for treatment of gastric cancer. EMEA review began on 13 August 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.

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OTHER INFORMATION ON THE ORPHAN MEDICINAL PRODUCT DESIGNATION

List of question

The COMP adopted one list of question on initial application. This application will be discussed again at the next COMP plenary meeting prior to adoption of the opinion.

Oral hearings

Four oral hearings took place following written responses to the lists of questions.

Withdrawal of applications for orphan medicinal product designation

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

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 DECEMBER 2007 COMP PLENARY MEETING

- The eighty-fifth meeting of the COMP will be held on 5-6 December 2007.

ORGANISATIONAL MATTERS

The main topics addressed during the November 2007 COMP meeting related to:

- The appointment of Prof. Josep Torrent Farnell as the COMP member representative in the DG SANCO Rare Disease Task Force.
- 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
2007	107	92	17	1	67
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 OCTOBER 2007 COMP PLENARY REPORT BY THE EUROPEAN COMMISSION

Active substance	4-[3,5-bis(trimethylsilyl)benzamido]benzoic acid
Sponsor	Quitiles Ireland Ltd
Orphan Indication	Treatment of hepatocellular carcinoma
COMP Opinion date	26/09/2007
Orphan Designation date	22/10/2007

Active substance	Alvocidib
Sponsor	Sanofi Aventis
Orphan Indication	Treatment of chronic lymphocytic leukaemia
COMP Opinion date	26/09/2007
Orphan Designation date	23/10/2007

Active substance	Amonafide L-malate
Sponsor	INC Research UK Ltd
Orphan Indication	Treatment of acute myeloid leukaemia
COMP Opinion date	26/09/2007
Orphan Designation date	22/10/2007

Active substance	Adenovirus associated viral vector serotype 4 containing the human RPE65 gene
Sponsor	Centre Hospitalier de Nantes
Orphan Indication	Treatment of Leber’s congenital amaurosis
COMP Opinion date	26/09/2007
Orphan Designation date	22/10/2007

Active substance	Ciclosporin
Sponsor	Novagali Pharma SA
Orphan Indication	Prevention of corneal graft rejection
COMP Opinion date	26/09/2007
Orphan Designation date	22/10/2007

Active substance	Iodine (^{131}I) chlorotoxin
Sponsor	The Weinberg Group L.L.C.

Orphan Indication	Treatment of glioma
COMP Opinion date	26/09/2007
Orphan Designation date	22/10/2007

Active substance	Isofagomine tartrate
Sponsor	Amicus Therapeutics UK Limited
Orphan Indication	Treatment of Gaucher Disease
COMP Opinion date	26/09/2007
Orphan Designation date	23/10/2007

Active substance	Mercaptopurine (oral liquid)
Sponsor	Only for Children Pharmaceuticals
Orphan Indication	Treatment of acute lymphoblastic leukaemia
COMP Opinion date	26/09/2007
Orphan Designation date	22/10/2007

Active substance	Methotrexate (oral liquid)
Sponsor	Only for Children Pharmaceuticals
Orphan Indication	Treatment of acute lymphoblastic leukaemia
COMP Opinion date	26/09/2007
Orphan Designation date	24/10/2007

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

Active substance	Invented name	Sponsor/applicant	EU Designation Number	Designated Orphan Indication
Anti-epidermal growth factor receptor antibody h-R3	Theraloc	Oncoscience AG	EU/3/04/220	Treatment of glioma