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

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COMMITTEE FOR ORPHAN MEDICINAL PRODUCTS
DECEMBER 2007 PLENARY MEETING
MONTHLY REPORT

The Committee for Orphan Medicinal Products (COMP) held its eighty-fifth plenary meeting on 5 December 2007. The Committee welcomed Dr Saleh as the new COMP member for Romania. The Committee also welcomed Dr Mey Wang and Dr Rou-Fang Chen, visiting experts from Taiwan.

ORPHAN MEDICINAL PRODUCT DESIGNATION

The COMP adopted 5 positive opinions recommending the following medicines for designation as orphan medicinal products to the European Commission:

- **(manganese, dichloro [(4aR, 13aR, 17aR, 21aR)-1, 2, 3, 4, 4a, 5, 6, 12, 13, 13a, 14, 15, 16, 17, 17a, 18, 19, 20, 21, 21a-eicosahydro-11, 7-nitrilo-7H-dibenzo[b,h] [1,4,7,10] tetraazacycloheptadecine-κN5, κN13, κN18, κN21, κN22]-),** from Celtic Bio-Pharma Services Ltd, for prevention of oral mucositis in head and neck cancer patients undergoing radiation therapy. EMEA review began on 10 September 2007 with an active review time of 88 days.
- **(R)-2-Methyl-6-nitro-2-{4-[4-(4-trifluoromethoxyphenoxy)piperidin-1-yl]phenoxymethyl}-2,3-dihydroimidazo[2,1-b]oxazole,** from Otsuka Pharmaceutical Europe Ltd, for treatment of tuberculosis. EMEA review began on 15 October 2007 with an active review time of 53 days.
- **Iodine (^{131}I) iobenguane,** from Molecular Insight Limited, for treatment of neuroblastoma. EMEA review began on 15 October 2007 with an active review time of 53 days.
- **Lutetium (^{177}Lu)-N-[(4,7,10-Tricarboxymethyl-1,4,7,10-tetraazacyclododec-1-yl)acetyl]-D-phenylalanyl-L-cysteinyl-L-tyrosyl-D-tryptophanyl-L-lysyl-L-threoninyl-L-cysteinyl-L-threonine-cyclic(2-7)disulfide,** from BioSynthema Global Operations B.V, for treatment of gastro-entero-pancreatic neuroendocrine tumours. EMEA review began on 15 October 2007 with an active review time of 53 days.
- **N-(2-Amino-phenyl)-4-[(4-pyridin-3-yl-pyrimidin-2-ylamino)-methyl] benzamide,** from Pharmion Ltd, for treatment of acute myeloid leukaemia. EMEA review began on 15 October 2007 with an active review time of 53 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 4 lists of questions on initial applications. These applications will be discussed again at the next COMP plenary meeting prior to adoption of the opinion.

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Oral hearings

No oral hearing took place.

Withdrawals of applications for orphan medicinal product designation

The COMP noted that no application for orphan medicinal product designation was 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.

Article 5 (12) of Regulation (EC) No 141/2000 of the European Parliament and of the Council

In line with its responsibility to review whether or not a designated orphan medicinal product still fulfils the designation criteria prior to the granting of a marketing authorisation, the COMP adopted one opinion via written procedure recommending to the European Commission that the following orphan medicinal product be kept in the Community registry of orphan medicinal products:

- **Nilotinib,** from Novartis Europharm Limited, for treatment of chronic myeloid leukaemia

UPCOMING MEETINGS FOLLOWING THE DECEMBER 2007 COMP PLENARY MEETING

- The eighty-sixth meeting of the COMP will be held on 9-10 January 2007.

ORGANISATIONAL MATTERS

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

- The appointment of Dr Flavia Saleh as the new COMP member from Romania.
- Discussion on the final draft for public consultation of the Communication from the Commission Regarding European Action in the Field of Rare Diseases.
- One Protocol Assistance letter was adopted.

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

[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)

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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	117	97	17	1	77
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 NOVEMBER 2007 COMP PLENARY REPORT BY THE EUROPEAN COMMISSION

Active substance	4-ethoxy-2-(piperazin-1-yl)-7-(pyridin-4-yl)-5H-pyrimido[5,4-b]indol
Sponsor	Curacyte Discovery GmbH
Orphan Indication	Treatment of chronic lymphocytic leukaemia
COMP Opinion date	12/09/2007
Orphan Designation date	14/11/2007

Active substance	Adenovirus associated viral vector serotype 4 containing the human RPE65 gene
Sponsor	Centre Hospitalier Universitaire de Nantes
Orphan Indication	Treatment of retinitis pigmentosa
COMP Opinion date	12/09/2007
Orphan Designation date	14/11/2007

Active substance	Azacitidine
Sponsor	Pharmion Limited
Orphan Indication	Treatment of acute myeloid leukaemia
COMP Opinion date	10/10/2007
Orphan Designation date	29/11/2007

Active substance	Ciclosporin
Sponsor	Novagali Pharma SA
Orphan Indication	Treatment of Herpes simplex virus stromal keratitis
COMP Opinion date	12/09/2007
Orphan Designation date	29/10/2007

Active substance	Human autologous bone-forming cells derived from bone marrow stem cells
Sponsor	Bone Therapeutics SA
Orphan Indication	Treatment of non-traumatic osteonecrosis
COMP Opinion date	12/09/2007
Orphan Designation date	29/10/2007

Active substance	everolimus
Sponsor	Novartis Europharm Limited

Orphan Indication	Treatment of gastro-entero-pancreatic neuroendocrine tumours
COMP Opinion date	12/09/2007
Orphan Designation date	14/11/2007

Active substance	Interferon beta
Sponsor	Faron Pharmaceuticals Limited
Orphan Indication	Treatment of acute lung injury
COMP Opinion date	10/10/2007
Orphan Designation date	29/11/2007

Active substance	Interferon gamma
Sponsor	Foundation for Fatal Rare Diseases
Orphan Indication	Treatment of idiopathic pulmonary fibrosis
COMP Opinion date	12/09/2007
Orphan Designation date	29/10/2007

Active substance	Irinotecan 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	Polihexanide
Sponsor	S.I.F.I. Società Industria Farmaceutica Italiana S.p.A.
Orphan Indication	Treatment of acanthamoeba keratitis
COMP Opinion date	26/09/2007
Orphan Designation date	14/11/2007

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

Active substance	Invented name	Sponsor/applicant	EU Designation Number	Designated Orphan Indication
Tetrahydrobiopterin	Sapropterin Merck	Merck KGaA	EU/3/04/199	Treatment of hyperphenylalaninemia
Recombinant megakaryopoiesis-stimulating protein	Nplate	Amgen Europe B.V.	EU/3/05/283	Treatment of idiopathic thrombocytopenic purpura
Suberolylanilide Hydroxamic acid	Vorinostat MSD	Merck Sharp & Dohme Limited	EU/3/04/205	Treatment of cutaneous T-cell lymphoma