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

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COMMITTEE FOR ORPHAN MEDICINAL PRODUCTS
FEBRUARY 2008 PLENARY MEETING
MONTHLY REPORT

The Committee for Orphan Medicinal Products (COMP) held its eighty-seventh plenary meeting on 5-6 February 2008.

The Committee welcomed both Mr. Antoni Montserrat from DG SANCO and Dr Ségolène Aymé from Orphanet and chair of the Rare Diseases Task Force.

Mr. Antoni Montserrat was invited by the Committee to give a presentation on the Communication from the Commission Regarding European Action in the Field of Rare Diseases in the context of its public consultation. The Committee congratulated the European Commission for initiating the drafting of the Communication. The Committee's contribution to the public consultation will be sent to the European Commission.

Dr Ségolène Aymé was invited to give a presentation on the epidemiological data collected on rare diseases for the 'Prevalence of rare diseases: a bibliographical survey' from Orphanet dated October 2007.

ORPHAN MEDICINAL PRODUCT DESIGNATION

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

- **Allogeneic human umbilical cord tissue-derived cells,** from Centocor, B.V., for treatment of retinitis pigmentosa. EMEA review began on 7 December 2007 with an active review time of 62 days.
- **Ammonium tetrathiomolybdate,** from JJGConsultancy Ltd, for treatment of Wilson's disease. EMEA review began on 7 December 2007 with an active review time of 62 days.
- **Amrubicin hydrochloride,** from Pharmion Ltd, for treatment of small cell lung cancer. EMEA review began on 7 December 2007 with an active review time of 62 days.
- **Ascorbic acid,** from Murigenetics SAS, for treatment of Charcot-Marie-Tooth disease type 1A. EMEA review began on 9 November 2007 with an active review time of 90 days.
- **Autologous urothelial and smooth muscle cells,** from Choice Pharma Limited, for treatment of spina bifida. EMEA review began on 7 December 2007 with an active review time of 62 days.
- **Chimeric antibody to mesothelin,** from Chiltern International Limited, for treatment of pancreatic cancer. EMEA review began on 7 December 2007 with an active review time of 62 days.
- **Filgrastim,** from Sygnis Bioscience GmbH & Co. KG, for treatment of amyotrophic lateral sclerosis. EMEA review began on 9 November 2007 with an active review time of 90 days.
- **Humanised monoclonal antibody to the folate receptor alpha,** from Chiltern International Limited, for treatment of ovarian cancer. EMEA review began on 7 December 2007 with an active review time of 62 days.

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- **Recombinant human monoclonal antibody against transforming growth factor beta-1, 2 and 3,** from Genzyme Europe BV, for treatment of idiopathic pulmonary fibrosis. EMEA review began on 9 November 2007 with an active review time of 90 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 three 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

Two oral hearings took place.

Withdrawals of applications for orphan medicinal product designation

The COMP noted that three of the 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.

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 recommending to the European Commission that the following orphan medicinal products be kept in the Community registry of orphan medicinal product:

- **Thalidomide,** from Pharmion Ltd., for treatment of multiple myeloma

UPCOMING MEETINGS FOLLOWING THE FEBRUARY 2008 COMP PLENARY MEETING

- EU Task Force on Rare Diseases 28 February 2008.
- The eighty-eighth meeting of the COMP will be held on 4-5 March 2008.

[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)

ORGANISATIONAL MATTERS

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

- Discussion and revision of the COMP Work Programme.
- Discussion on the Communication from the Commission Regarding European Action in the Field of Rare Diseases under public consultation with DG SANCO
- Discussion on the report 'Prevalence of rare diseases: a bibliographical survey' from Orphanet dated October 2007
- Four 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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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	6	13	5	-	5
2007	125	97	19	1	98
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 JANUARY 2008 COMP PLENARY REPORT BY THE EUROPEAN COMMISSION

Active substance	(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]-)
Sponsor	Celtic Bio-Pharma Services Ltd
Orphan Indication	Prevention of oral mucositis in head and neck cancer patients undergoing radiation therapy
COMP Opinion date	05/12/2007
Orphan Designation date	31/01/2008

Active substance	(R)-2-Methyl-6-nitro-2-{4-[4-(4-trifluoromethoxyphenoxy) piperidin-1-yl]phenoxymethyl}-2,3-dihydroimidazo[2,1-b]oxazole
Sponsor	Otsuka Pharmaceutical Europe Ltd
Orphan Indication	Treatment of tuberculosis
COMP Opinion date	05/12/2007
Orphan Designation date	01/02/2008

Active substance	Heterologous human adult liver derived stem cells
Sponsor	Prof. Etienne Sokal
Orphan Indication	Treatment of Crigler-Najjar syndrome
COMP Opinion date	30/10/2007
Orphan Designation date	29/12/2007

Active substance	Lutetium (177Lu)-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
Sponsor	BioSynthema Global Operations B.V
Orphan Indication	Treatment of gastro-entero-pancreatic neuroendocrine tumours
COMP Opinion date	05/12/2007
Orphan Designation date	31/01/2008

Active substance	Iodine (131I) iobenguane
Sponsor	Molecular Insight Limited
Orphan Indication	Treatment of neuroblastoma
COMP Opinion date	05/12/2007
Orphan Designation date	31/01/2008

Active substance	Methyl 4,6-diamino-2-[1-(2-fluorobenzyl)-1H-pyrazolo[3,4-b]pyridine-3-yl]-5-pyrimidinyl(methyl)carbamate
Sponsor	Bayer HealthCare AG
Orphan Indication	Treatment of pulmonary arterial hypertension including treatment of chronic thromboembolic pulmonary hypertension
COMP Opinion date	08/11/2007
Orphan Designation date	20/12/2007

Active substance	N-(2-amino-phenyl)-4-[(4-pyridin-3-yl-pyrimidin-2-ylamino)-methyl] benzamide
Sponsor	Pharmion Ltd
Orphan Indication	Treatment of acute myeloid leukaemia
COMP Opinion date	05/12/2007
Orphan Designation date	31/01/2008

Active substance	N-[4-(3-amino-1H-indazol-4 yl)phenyl]-N'-(2-fluoro-5-methylphenyl) urea
Sponsor	Abbott Laboratories Limited
Orphan Indication	Treatment of hepatocellular carcinoma
COMP Opinion date	08/11/2007
Orphan Designation date	20/12/2007

Active substance	Tegafur, grimeracil, oteracil potassium
Sponsor	Sanofi Aventis
Orphan Indication	Treatment of gastric cancer
COMP Opinion date	08/11/2007
Orphan Designation date	20/12/2007

DESIGNATED ORPHAN MEDICINAL PRODUCTS THAT HAVE BEEN SUBJECT OF A NEW COMMUNITY MARKETING AUTHORISATION APPLICATION UNDER THE CENTRALISED PROCEDURE SINCE THE JANUARY 2008 COMP MONTHLY REPORT

Active substance	Invented name	Sponsor/applicant	EU Designation Number	Designated Orphan Indication
Azacitidine	Vidaza	Pharmion Ltd.	EU/3/01/084	Treatment of myelodysplastic syndromes