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

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COMMITTEE FOR ORPHAN MEDICINAL PRODUCTS
MAY 2008 PLENARY MEETING
MONTHLY REPORT

The Committee for Orphan Medicinal Products (COMP) held its ninetieth plenary meeting on 13-14 May 2008.

The Committee welcomed Dr Tim Coté, director of the FDA's Office of Orphan Drug Development. The Committee discussed with him the future collaboration in the field of rare diseases, particularly the encouragement for cross submissions for orphan designation to the FDA and EMEA, the strengthening of communication between agencies, and the future collaboration for annual reports on development. Dr Coté presented the FDA initiative to incentivise submissions for orphan designation for tropical diseases.

ORPHAN MEDICINAL PRODUCT DESIGNATION

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

- **Vincristine sulphate liposomes**, from QuadraMed Limited, for treatment of acute lymphoblastic leukaemia. EMEA review began on 15 February 2008 with an active review time of 89 days.
- **Beraprost sodium (modified release tablet),** from Lung Rx Limited, for treatment of pulmonary arterial hypertension. EMEA review began on 14 March 2008 with an active review time of 61 days.
- **N-(2,4-Di-tert-butyl-5-hydroxyphenyl)-1,4-dihydro-4-oxoquinoline-3-carboxamide,** from Voisin Consulting S.A.R.L., for treatment of cystic fibrosis**.** EMEA review began on 14 March 2008 with an active review time of 61 days
- **Sapacitabine,** from Cyclacel Limited, for treatment of myelodysplastic syndrome**.** EMEA review began on 14 March 2008 with an active review time of 61 days.
- **Sapacitabine,** from Cyclacel Limited, for treatment of acute myeloid leukaemia**.** EMEA review began on 14 March 2008 with an active review time of 61 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.

Since January 2008, 66% of the positive opinions have been adopted after the first discussion of the application, therefore without necessity of an oral explanation by the sponsor. Up to May, the average time to reach a positive opinion in 2008 is 66.25 days.

OTHER INFORMATION ON THE ORPHAN MEDICINAL PRODUCT DESIGNATION

Lists of questions

The COMP adopted six 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

One oral hearing took place.

Withdrawals of applications for orphan medicinal product designation

The COMP noted that one 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 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:

- **Icatibant acetate,** from Jerini AG for treatment of angioedema.

UPCOMING MEETINGS FOLLOWING THE APRIL 2008 COMP PLENARY MEETING

- The ninety one meeting of the COMP will be held on 10-11 June 2008.
- The Informal COMP meeting will be held on the 26-27 May 2008 in Bled, Slovenia.

ORGANISATIONAL MATTERS

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

- The Committee adopted the final agenda for the Informal COMP meeting on the 26-27 May 2008 in Bled, Slovenia.
- 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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[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
2008	35	32	15	-	24
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

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MEDICINAL PRODUCTS GRANTED A COMMUNITY DESIGNATION AS ORPHAN MEDICINAL PRODUCT SINCE THE APRIL 2008 COMP PLENARY REPORT BY THE EUROPEAN COMMISSION

Active substance	Ammonium tetrathiomolybdate
Sponsor	JJGConsultancy Ltd - UK
Orphan Indication	Treatment of Wilson's disease
COMP Opinion date	6 February 2008
Orphan Designation date	1 April 2008

Active substance	Amrubicin hydrochloride
Sponsor	Pharmion Ltd - UK
Orphan Indication	Treatment of small cell lung cancer
COMP Opinion date	6 February 2008
Orphan Designation date	2 April 2008

Active substance	Humanised monoclonal antibody to the folate receptor alpha
Sponsor	Chiltern International Limited - United Kingdom
Orphan Indication	Treatment of ovarian cancer
COMP Opinion date	6 February 2008
Orphan Designation date	1 April 2008

Active substance	Allogeneic human umbilical cord tissue-derived cells (INN)
Sponsor	Centocor, B.V. - The Netherlands
Orphan Indication	Treatment of retinitis pigmentosa
COMP Opinion date	6 February 2008
Orphan Designation date	1 April 2008

Active substance	Recombinant human monoclonal antibody against transforming growth factor beta-1, 2 and 3
Sponsor	Genzyme BV - The Netherlands
Orphan Indication	Treatment of idiopathic pulmonary fibrosis
COMP Opinion date	6 February 2008
Orphan Designation date	1 April 2008

Active substance	Filgrastim
Sponsor	Sygnis Bioscience GmbH & Co. KG - Germany

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Orphan Indication	Treatment of amyotrophic lateral sclerosis
COMP Opinion date	6 February 2008
Orphan Designation date	1 April 2008

Active substance	Ascorbic acid
Sponsor	Murigenetics SAS - France
Orphan Indication	Treatment of Charcot-Marie-Tooth disease type 1A
COMP Opinion date	6 February 2008
Orphan Designation date	1 April 2008

Active substance	Autologous urothelial and smooth muscle cells
Sponsor	Choice Pharma Limited - UK
Orphan Indication	Treatment of spina bifida
COMP Opinion date	6 February 2008
Orphan Designation date	17 March 2008

Active substance	Omigapil maleate
Sponsor	Santhera Pharmaceuticals (Deutschland) AG - Germany
Orphan Indication	Treatment of congenital muscular dystrophy with merosin (laminin alpha 2) deficiency
COMP Opinion date	4 March 2008
Orphan Designation date	8 May 2008

Active substance	Omigapil maleate
Sponsor	Santhera Pharmaceuticals (Deutschland) GmbH - Germany
Orphan Indication	Treatment of congenital muscular dystrophy with collagen VI deficiency (Ullrich Syndrome and Bethlem Myopathy)
COMP Opinion date	4 March 2008
Orphan Designation date	8 May 2008

Active substance	[Nle^{4}, D-Phe^{7}]-alfa-melanocyte stimulating hormone
Sponsor	Clinuvel UK Limited - UK
Orphan Indication	Treatment of erythropoietic protoporphyria
COMP Opinion date	4 March 2008
Orphan Designation date	8 May 2008

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Active substance	[Nle^4, D-Phe^7]-alfa-melanocyte stimulating hormone
Sponsor	Clinuvel UK Limited - UK
Orphan Indication	Treatment of congenital erythropoietic porphyria
COMP Opinion date	4 March 2008
Orphan Designation date	8 May 2008

Active substance	Ribonucleotide reductase R2 specific phosphorothioate oligonucleotide
Sponsor	Dr Ulrich Granzer - Germany
Orphan Indication	Treatment of acute myeloid leukemia
COMP Opinion date	4 March 2008
Orphan Designation date	8 May 2008

Active substance	Sarsasapogenin
Sponsor	Phytopharm plc - UK
Orphan Indication	Treatment of amyotrophic lateral sclerosis
COMP Opinion date	4 March 2008
Orphan Designation date	8 May 2008

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