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

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COMMITTEE FOR ORPHAN MEDICINAL PRODUCTS
JUNE 2008 PLENARY MEETING
MONTHLY REPORT

The Committee for Orphan Medicinal Products (COMP) held its ninety-first plenary meeting on 10-11 June 2008. The Committee discussed new ways to improve the work of the COMP, namely a revision of the structure of the reports and ways to collaborate with the Paediatric Committee for issues of common interest. The COMP agreed to discuss this further at the next meeting and the next COMP Informal meeting.

ORPHAN MEDICINAL PRODUCT DESIGNATION

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

- **(-)-(2R)-3-(2-hydroxymethylindanyl-4-oxy)-phenyl-4,4,4-trifluorobutane-1-sulfonate ,** KeyNeurotek Pharmaceuticals AG, treatment of moderate and severe closed traumatic brain injury. EMEA review began on 14 March 2008 with an active review time of 90 days.
- **Bosentan,** Actelion Research Limited, treatment of idiopathic pulmonary fibrosis. EMEA review began on 11 April 2008 with an active review time of 62 days.
- **Donor lymphocyte preparation depleted of functional alloreactive T-cells,** Kiadis Pharma Netherlands B.V., Prevention of Graft-versus-Host disease. EMEA review began on 11 April 2008 with an active review time of 62 days.
- **Recombinant derivative of C3 transferase,** Triskel EU Services, treatment of traumatic spinal cord injury. EMEA review began on 14 March 2008 with an active review time of 90 days.
- **Topotecan hydrochloride (liposomal),** Dr Matthias Luz, treatment of glioma. EMEA review began on 11 April 2008 with an active review time of 62 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.

Oral hearings

Four oral hearings took place.

Withdrawals of applications for orphan medicinal product designation

The COMP noted that 4 of applications for orphan medicinal product designation were withdrawn.

7 Westferry Circus, Canary Wharf, London, E14 4HB, UK
Tel. (44-20) 74 18 84 00 Fax (44-20) 74 18 86 13
E-mail: mail@emea.europa.eu http://www.emea.europa.eu

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 <MONTH/YEAR> COMP PLENARY MEETING

- The ninety-second meeting of the COMP will be held on 8-9 July 2008.

ORGANISATIONAL MATTERS

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

- The appointment of Mr Agnis Zvaigzne as the new COMP member from Latvia.
- Discussion on work improvements of the COMP was presented.
- Discussion of the one year experience of the Paediatric Regulation
- 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

For further information, please contact:
Martin Harvey Allchurch, EMEA press officer
Tel. (+44-20) 74 18 84 27
E-mail: press@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)

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	37	19	-	32
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 MAY 2008 COMP PLENARY REPORT BY THE EUROPEAN COMMISSION

Active substance	Alpha-1 proteinase inhibitor (for inhalation use)
Sponsor	Talecris Biotherapeutics GmbH
Orphan Indication	Treatment of congenital alpha-1 antitrypsin deficiency
COMP Opinion date	08/04/2008
Orphan Designation date	03/06/2008

Active substance	Anti-von Willebrand Aptamer
Sponsor	FGK Representative Service GmbH
Orphan Indication	Treatment of thrombotic thrombocytopenic purpura
COMP Opinion date	08/04/2008
Orphan Designation date	03/06/2008

Active substance	Carfilzomib
Sponsor	Interface International Consultancy Limited
Orphan Indication	Treatment of multiple myeloma
COMP Opinion date	08/04/2008
Orphan Designation date	03/06/2008

Active substance	NGR-human Tumour Necrosis Factor
Sponsor	MolMed S.p.A.
Orphan Indication	Treatment of malignant mesothelioma
COMP Opinion date	08/04/2008
Orphan Designation date	03/06/2008

Active substance	Nimotuzumab
Sponsor	Oncoscience AG
Orphan Indication	Treatment of pancreatic cancer
COMP Opinion date	08/04/2008
Orphan Designation date	03/06/2008

Active substance	Pegylated recombinant factor VIIa
Sponsor	Novo Nordisk A/S

Orphan Indication	Treatment of haemophilia A
COMP Opinion date	08/04/2008
Orphan Designation date	04/06/2008

Active substance	Pegylated recombinant factor VIIa
Sponsor	Novo Nordisk A/S
Orphan Indication	Treatment of haemophilia B
COMP Opinion date	08/04/2008
Orphan Designation date	03/06/2008

Active substance	Recombinant fusion protein of circulary-permuted IL-4 and pseudomonas exotoxin A, [IL-4(38-37)-PE38KDEL]
Sponsor	Gregory Fryer Associates Ltd
Orphan Indication	Treatment of glioma
COMP Opinion date	08/04/2008
Orphan Designation date	03/06/2008

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

Active substance	Invented name	Sponsor/applicant	EU Designation Number	Designated Orphan Indication
Caffeine citrate	Caffeine citrate Chiesi	Chiesi Farmaceutici S.P.A.	EU/3/03/132	Treatment of primary apnoea of premature newborns