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

9 April 2008
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COMMITTEE FOR ORPHAN MEDICINAL PRODUCTS
APRIL 2008 PLENARY MEETING
MONTHLY REPORT

The Committee for Orphan Medicinal Products (COMP) held its eighty-ninth plenary meeting on 8 April 2008.

ORPHAN MEDICINAL PRODUCT DESIGNATION

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

- **Anti-von Willebrand Aptamer,** from FGK Representative Service GmbH, for treatment of thrombotic thrombocytopenic purpura. EMEA review began on 11 January 2008 with an active review time of 89 days.
- **Alpha-1 proteinase inhibitor (for inhalation use),** from Talecris Biotherapeutics GmbH, for treatment of congenital alpha-1 antitrypsin deficiency. EMEA review began on 15 February 2008 with an active review time of 54 days.
- **Carfilzomib,** from Interface International Consultancy Ltd, for treatment of multiple myeloma. EMEA review began on 15 February 2008 with an active review time of 54 days.
- **NGR-human Tumour Necrosis Factor,** from MolMed S.p.A., for treatment of malignant mesothelioma. EMEA review began on 15 February 2008 with an active review time of 54 days.
- **Nimotuzumab,** from Oncoscience AG, for treatment of pancreatic cancer. EMEA review began on 15 February 2008 with an active review time of 54 days.
- **Pegylated recombinant factor VIIa,** from Novo Nordisk A/S, for treatment of haemophilia B. EMEA review began on 15 February 2008 with an active review time of 54 days.
- **Pegylated recombinant factor VIIa,** from Novo Nordisk A/S, for treatment of haemophilia A. EMEA review began on 15 February 2008 with an active review time of 54 days.
- **Recombinant fusion protein of circulary-permuted IL-4 and pseudomonas exotoxin A, [IL-4(38-37)-PE38KDEL],** from Gregory Fryer Associates Ltd, for treatment of glioma. EMEA review began on 15 February 2008 with an active review time of 54 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 two lists of questions on initial applications. These applications will be discussed again at the next COMP plenary meeting prior to adoption of the opinion.

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

Oral hearings

Two oral hearings took place.

Withdrawals of applications for orphan medicinal product designation

The COMP noted that three 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 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 APRIL 2008 COMP PLENARY MEETING

- The ninetieth meeting of the COMP will be held on 13-14 May 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:

- Discussion on the agenda for the Informal COMP meeting on the 26-27 May 2008 in Bled, Slovenia.
- 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	29	28	9	-	10
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 MARCH 2008 COMP PLENARY REPORT BY THE EUROPEAN COMMISSION

Active substance	Chimeric antibody to mesothelin
Sponsor	Chiltern International Limited
Orphan Indication	Treatment of pancreatic cancer
COMP Opinion date	06/02/2008
Orphan Designation date	17/03/2008