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

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COMMITTEE FOR ORPHAN MEDICINAL PRODUCTS
MAY 2009 PLENARY MEETING
MONTHLY REPORT

The Committee for Orphan Medicinal Products (COMP) held its 101th plenary meeting on 5 May 2009. The Committee designated for the first time an orphan medicinal product for treatment of Rett syndrome, a developmental disorder caused by mutations on the X chromosome. At the time of submission of the application for orphan designation, no satisfactory method had been authorised in the European Union for treatment of the condition.

ORPHAN MEDICINAL PRODUCT DESIGNATION

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

For the following medicines the EMEA review began on 6 February 2009 with an active review time of 89 days.

- **Chimeric-anti-interleukin-6 monoclonal antibody** for treatment of multiple myeloma, from Centocor, B.V.

For the following medicines the EMEA review began on 9 March 2009 with an active review time of 58 days.

- **Desipramine chlorhydrate** for treatment of Rett syndrome, from Targeon SAS
- **Murine monoclonal antibody to GD2** for treatment of neuroblastoma, from United Therapeutics Europe Ltd
- **Octreotide chloride** for treatment of acromegaly, from Camurus AB
- **Talampanel** for treatment of amyotrophic lateral sclerosis, from Teva Pharma GmbH

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 four lists of questions on initial applications. These applications will be discussed again at the next COMP plenary meeting prior to adoption of the opinion.

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**.

[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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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(s):

- **Caffeine citrate (Nymusa),** for treatment of primary apnoea of premature newborns, from Chiesi Farmaceutici S.P.A

UPCOMING MEETINGS FOLLOWING THE MAY 2009 COMP PLENARY MEETING

- The 102nd meeting of the COMP will be held on 3-4 June 2009.

OTHER MATTERS

The main topics addressed during the May 2009 COMP meeting related to:

- Discussion on the agenda topics for the Informal COMP meeting to be held on 1-2 October 2009 in Stockholm.
- Discussion on the topics presented at the Rare Disease Task Force meeting held on 30 April 2009 in Luxembourg.
- Discussion on the ongoing EMEA transparency project
- Two 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
2009	41	34	5	-	28
2008	119	86	31	1	73
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 APRIL 2009 COMP PLENARY REPORT BY THE EUROPEAN COMMISSION

Active substance	2,2-dimethylbutyric acid, sodium salt
Sponsor	Isabelle Ramirez
Orphan Indication	Treatment of sickle cell disease
COMP Opinion date	09/02/2009
Orphan Designation date	18/03/2009

Active substance	N-(5-tert-Butylisoxazol-3-yl)-N'-{4-[7-(2-(morpholin-4-yl)ethoxy) imidazo[2,1-b][1,3]benzothiazol-2-yl]phenyl}urea di-hydrochloride salt
Sponsor	Ambit Europe Limited
Orphan Indication	Treatment of acute myeloid leukaemia
COMP Opinion date	09/02/2009
Orphan Designation date	23/03/2009

Active substance	(R)-3-(4-(7H-pyrrolol[2,3-d]pyrimidin-4-yl)-1Hpyrazol-1-yl)-3-cyclopentylpropanenitrile phosphate
Sponsor	Incyte Corporation Ltd
Orphan Indication	Treatment of myelofibrosis secondary to polycythemia vera or essential thrombocythemia
COMP Opinion date	09/02/2009
Orphan Designation date	03/04/2009