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

4 March 2008
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COMMITTEE FOR ORPHAN MEDICINAL PRODUCTS
MARCH 2008 PLENARY MEETING
MONTHLY REPORT

The Committee for Orphan Medicinal Products (COMP) held its eighty-eighth plenary meeting on 4 March 2008. The Committee welcomed a new observer, Dr Maria Mavris who works for the European Organisation for Rare Diseases.

ORPHAN MEDICINAL PRODUCT DESIGNATION

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

- **[Nle4, D-Phe7]-alpha-melanocyte stimulating hormone**, from Clinuvel UK Limited, for treatment of erythropoietic protoporphyria. EMEA review began on 7 December 2007 with an active review time of 89 days.
- **[Nle4, D-Phe7]-alpha-melanocyte stimulating hormone**, from Clinuvel UK Limited, for treatment of congenital erythropoietic porphyria. EMEA review began on 7 December 2007 with an active review time of 89 days.
- **Omigapil maleate,** from Santhera Pharmaceuticals (Deutschland) GmbH, for treatment of congenital muscular dystrophy with collagen VI deficiency (Ullrich Syndrome and Bethlem Myopathy). EMEA review began on 7 December 2007 with an active review time of 89 days.
- **Omigapil maleate,** from Santhera Pharmaceuticals (Deutschland) GmbH, for treatment of congenital muscular dystrophy with merosin (laminin alpha 2) deficiency. EMEA review began on 7 December 2007 with an active review time of 89 days.
- **Ribonucleotide reductase R2 specific phosphorothioate oligonucleotide,** from Dr Ulrich Granzer, for treatment of acute myeloid leukaemia. EMEA review began on 11 January 2008 with an active review time of 54 days.
- **Sarsapogenin,** from Phytopharm plc, for treatment of amyotrophic lateral sclerosis. EMEA review began on 11 January 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 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.

Oral hearings

Three oral hearings took place.

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Withdrawal of application 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:

- **Ambrisentan,** from Glaxo Group Limited for treatment of pulmonary arterial hypertension and chronic thromboembolic pulmonary hypertension.

UPCOMING MEETINGS FOLLOWING THE MARCH 2008 COMP PLENARY MEETING

- The eighty-ninth meeting of the COMP will be held on 8-9 April 2008.

ORGANISATIONAL MATTERS

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

- Discussion and adoption on the revised Appeal Procedure for Orphan Medicinal Product Designation which is provided in **Annex 3**[2].
- Discussion on the EU Task Force on Rare Diseases meeting on the 28 February 2008.
- 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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[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)

[2] Second and the eleventh bullet points had been corrected

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	18	19	6	-	9
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 FEBRUARY 2008 COMP PLENARY REPORT BY THE EUROPEAN COMMISSION

Active substance	Heterologous human adult liver derived stem cells
Sponsor	Prof. Etienne Sokal
Orphan Indication	Treatment of ornithine transcarbamylase deficiency
COMP Opinion date	10/01/2008
Orphan Designation date	04/02/2008

Active substance	Lumiliximab
Sponsor	Biogen Idec Limited
Orphan Indication	Treatment of chronic lymphocytic leukaemia
COMP Opinion date	10/01/2008
Orphan Designation date	04/02/2008

Active substance	Recombinant human monoclonal antibody to human IL-1beta of the IgG1/K class
Sponsor	Novartis Europharm Limited
Orphan Indication	Treatment of systemic-onset juvenile idiopathic arthritis
COMP Opinion date	10/01/2008
Orphan Designation date	04/02/2008

Active substance	Tretazicar
Sponsor	Morvus technology Agency
Orphan Indication	Treatment of visceral leishmaniasis
COMP Opinion date	10/01/2008
Orphan Designation date	04/02/2008

Procedural advice on appeal procedure for Orphan Medicinal Product Designation

Legal Basis

In accordance with Article 5.7, Regulation (EC) No 141/2000 of 16 December 1999, where the opinion of the Committee for Orphan Medicinal Products is that an application does not satisfy the criteria for orphan medicinal product designation, the Agency shall forthwith inform the sponsor. Within 90 days of the receipt of the opinion, the sponsor may submit detailed grounds for appeal, which the Agency shall refer to the Committee. The Committee shall consider whether its opinion should be revised at the following meeting.

Appeal Procedure

- Upon adoption of an opinion on orphan medicinal product designation, the EMEA will forward the opinion to the sponsor together with a copy of the COMP Summary Report.
- The sponsor may inform the Agency of any intent to appeal, without delay after receipt of the opinion, by giving written notice to the EMEA.
- The COMP will appoint a new COMP co-ordinator for the appeal procedure. If necessary, the COMP may appoint additional experts.
- Detailed grounds for appeal must be submitted by the sponsor within 90 days of receipt of the opinion. The grounds for appeal should be submitted to the EMEA (1 paper copy + 2 electronic copies as CD-Rom).
- The EMEA will refer the grounds for appeal to the COMP immediately after receipt.
- The sponsor will be invited to an oral explanation before the COMP at the meeting following the receipt of the grounds.
- An ad-hoc expert meeting may be convened, as necessary.
- The EMEA co-ordinator, in association with the COMP co-ordinator, will update the COMP Summary Report. The revised Summary Report will be circulated for comments to COMP members and appointed expert(s) by the EMEA.
- The COMP, at the first meeting following the sponsor's submission of the grounds for appeal, having reviewed the detailed grounds for appeal and having heard the oral explanation of the sponsor, will consider whether its opinion should be revised and will adopt a final COMP opinion. Where possible the expert(s) involved in the application will be invited to attend the COMP discussion.
- The EMEA will forward the final opinion to the Commission and the sponsor.
- The Decision will be adopted by the Commission, within 30 days of its receipt of the final opinion. Once the decision has been issued, a public summary of opinion will be published on the EMEA website.
- Upon a favourable Decision by the Commission, the designated medicinal product shall be entered in the Community Register of Orphan Medicinal Products.