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

10 September 2008
Doc. Ref.: EMEA/COMP/479052/2008

COMMITTEE FOR ORPHAN MEDICINAL PRODUCTS
SEPTEMBER 2008 PLENARY MEETING
MONTHLY REPORT

The Committee for Orphan Medicinal Products (COMP) held its ninety-third plenary meeting on 9-10 September 2008. The Committee welcomed Dr Ulla Nähri, who will be working with COMP-EMEA activities with Ms Claire Scharf-Kröner at the European Commission, DG Enterprise, Pharmaceuticals.

ORPHAN MEDICINAL PRODUCT DESIGNATION

The COMP adopted 11 positive opinions recommending the following medicines for designation as orphan medicinal products to the European Commission:

- **(R)-3-(4-(7H-pyrrolo[2,3-d]pyrimidin-4-yl)-1H-pyrazol-1-yl)-3-cyclopentylpropanenitrile phosphate,** from Incyte Corporation Ltd, for treatment of chronic idiopathic myelofibrosis. EMEA review began on 11 July 2008 with an active review time of 62 days.
- **Adeno-associated viral vector containing the human alpha-sarcoglycan gene,** from Généthon, for treatment of alpha-sarcoglycanopathy. EMEA review began on 11 July 2008 with an active review time of 62 days.
- **Autologous urothelial and smooth muscle cells,** from Choice Pharma Limited, for treatment of spinal cord injury. EMEA review began on 11 July 2008 with an active review time of 62 days.
- **Carglumic acid,** from Orphan Europe SARL, for treatment of propionic acidaemia. EMEA review began on 11 July 2008 with an active review time of 62 days.
- **Carglumic acid,** from Orphan Europe SARL, for treatment of isovaleric acidaemia. EMEA review began on 11 July 2008 with an active review time of 62 days.
- **Carglumic acid,** from Orphan Europe SARL, for treatment of methylmalonic acidaemia. EMEA review began on 11 July 2008 with an active review time of 62 days.
- **Cysteamine hydrochloride,** from Orphan Europe SARL, for treatment of cystinosis. EMEA review began on 11 July 2008 with an active review time of 62 days.
- **Ex vivo expanded autologous human corneal epithelium containing stem cells,** from Chiesi Farmaceutici S.P.A., for treatment of corneal lesions, with associated corneal (limbal) stem cell deficiency, due to ocular burns. EMEA review began on 13 June 2008 with an active review time of 90 days.
- **Filgrastim,** from Sygnis Bioscience GmbH & Co.KG, for treatment of spinal cord injury. EMEA review began on 11 July 2008 with an active review time of 62 days.
- **Ofatumumab,** from Glaxo Group Limited, for treatment of chronic lymphocytic leukaemia. EMEA review began on 11 July 2008 with an active review time of 62 days.
- **Recombinant human heparan-N-sulfatase,** from Shire Pharmaceutical Development Limited, for treatment of mucopolysaccharidosis III, type A (Sanfilippo A syndrome). EMEA review began on 11 July 2008 with an active review time of 62 days.

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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.

Oral hearings

Three oral hearings took place.

Withdrawals of applications for orphan medicinal product designation

The COMP noted that two of 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 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.

The Committee was informed of the withdrawal from the Community Register of Sutent (sunitinib malate which was designated for treatment of renal cell carcinoma and treatment of advanced and/or metastatic renal cell carcinoma (MRCC) andmalignant gastrointestinal stromal tumours and authorised in the Community for the treatment of unresectable and/or metastatic malignant gastrointestinal stromal tumour (GIST) after failure of imatinib mesylate treatment due to resistance or intolerance.).

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 prouducts:

- **Histamine dihydrochloride** from EpiCept GmbH, for treatment of acute myeloid leukaemia

UPCOMING MEETINGS FOLLOWING THE SEPTEMBER 2008 COMP PLENARY MEETING

[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)

- The Informal COMP meeting will be held in Paris, France on 6-7 October 2008.
- The ninety-forth meeting of the COMP will be held on 7-8 October 2008.
- EPPOSI workshop will be held in Paris, France on the 16-17 October 2008

ORGANISATIONAL MATTERS

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

- Discussion on work improvements of the COMP to be further developed in particular in the ways to increase transparency.
- Debriefing of the ICORD meeting held in May 2008. Amongst other topics the need to improve international collaboration and to further support translational research were discussed at the ICORD meeting. Next ICORD meeting is planned to be held in Rome in 2009.
- Discussion with the significant benefit ad-hoc group on the revision on the draft Guideline on the 'Elements required to support the medical plausibility and the assumption of significant benefit for an orphan designation' (COMP/1527/03). The Guideline will be adopted at the next meeting and released for public consultation after adoption by the COMP.

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
2008	85	56	22	-	37
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 JULY 2008 COMP PLENARY REPORT BY THE EUROPEAN COMMISSION

Active substance	Beraprost sodium (modified release tablet)
Sponsor	Lung Rx Limited -
Orphan Indication	Treatment of pulmonary arterial hypertension
COMP Opinion date	14/05/2008
Orphan Designation date	08/07/2008

Active substance	N-(2,4-Di-tert-butyl-5-hydroxyphenyl)-1,4-dihydro-4-oxoquinoline-3-carboxamide
Sponsor	Voisin Consulting S.A.R.L.
Orphan Indication	Treatment of cystic fibrosis
COMP Opinion date	14/05/2008
Orphan Designation date	08/07/2008

Active substance	Sapacitabine
Sponsor	Cyclacel Limited
Orphan Indication	Treatment of myelodysplastic syndromes
COMP Opinion date	14/05/2008
Orphan Designation date	08/07/2008

Active substance	Sapacitabine
Sponsor	Cyclacel Limited
Orphan Indication	Treatment of acute myeloid leukaemia
COMP Opinion date	14/05/2008
Orphan Designation date	10/07/2008

Active substance	Vincristine sulphate liposomes
Sponsor	QuadraMed Limited
Orphan Indication	Treatment of acute lymphoblastic leukaemia
COMP Opinion date	14/05/2008
Orphan Designation date	08/07/2008

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

Active substance	Invented name	Sponsor/applicant	EU Designation Number	Designated Orphan Indication
Everolimus	Afinitor	Novartis Europharm Limited	EU/3/07/449	Treatment of renal cell carcinoma
Recombinant human IL-1beta of the IgG1/K class	Arcalyst	Regeneron UK	EU/3/07/439	Treatment of cryopirin-associated periodic syndromes (Familial Cold Urticaria Syndrome (FCUS), Muckle-Wells Syndrome (MWS), and Neonatal Onset Multisystem Inflammatory Disease (NOMID), also known as Chronic Infantile Neurological Cutaneous Articular Syndrome (CINCA))
Thiotepa	Tepadina	Adienne S.r.l	EU/3/06/424	Conditioning treatment prior to haematopoietic progenitor cell transplantation