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

London, 22 December 2008
Doc. Ref.: EMEA/666785/2008 Corr. 2*

COMMITTEE FOR ORPHAN MEDICINAL PRODUCTS
DECEMBER 2008 PLENARY MEETING
MONTHLY REPORT

The Committee for Orphan Medicinal Products (COMP) held its ninety-sixth meeting plenary meeting on 9-10 December 2008.

The Committee welcomed a delegation from Health Canada. Mr Wayne Lepine, Director of the Office of Pharmaceuticals Management Strategies presented to the COMP an update of their ongoing regulatory modernization activities and the current treatment of orphan drug products in Canada at both regulatory and reimbursement levels. Mr Lepine also noted that Canada was interested in learning more about the European approach to orphan drug products and in potential opportunities for EMEA-Health Canada co-operation in this area.

ORPHAN MEDICINAL PRODUCT DESIGNATION

The COMP adopted six 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 12 September 2008 with an active review time of 90 days.

- **Recombinant human hepatocarcinoma-intestine-pancreas / pancreatic associated protein,** from Alfact Innovation SAS, for treatment of acute liver failure.
- **Type I native bovine skin collagen,** from arGentis Autoimmune Europe Limited, for treatment of systemic sclerosis.

For the following medicines the EMEA review began on 13 October 2008 with an active review time of 59 days.

- **Adeno-associated viral vector serotype 5 containing the human *ABCA4* gene,** from Fondazione Telethon, for treatment of Stagardt's disease.
- **Cyclopropane-1,1-dicarboxylic acid [4-(6,7-dimethoxy-quinolin-4-yloxy)-phenyl]-amide (4-fluoro-phenyl)-amide, (L)-malate salt,** from PPD Global Ltd, for treatment of medullary thyroid carcinoma.
- **Recombinant human proinsulin,** from ProRetina Therapeutics S.L., for treatment of retinitis pigmentosa.
- **Yttrium (^{90}Y)-DOTA-radiolabelled humanized monoclonal antibody against mucin 1,** from Immunomedics GmbH, for treatment of pancreatic cancer.

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.

* Correction of the second paragraph and update of Annex 2 (pages 5-9)

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
© European Medicines Agency, 2008. Reproduction is authorised provided the source is acknowledged

OTHER INFORMATION ON THE ORPHAN MEDICINAL PRODUCT DESIGNATION

Lists of questions

The COMP adopted one list of questions on initial applications. This application will be discussed again at the next COMP plenary meeting prior to adoption of the opinion.

Oral hearings

Five oral hearings took place.

Withdrawals of applications for orphan medicinal product designation

The COMP noted that five 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[†] 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.

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 three opinions recommending to the European Commission to keep the following orphan medicinal products in the Community registry of orphan medicinal products:

- **Azacitidine,** from Celgene Europe Limited, for treatment myelodysplastic syndromes
- **Azacitidine,** from Celgene Europe Limited, for treatment acute myeloid leukaemia
- **Recombinant megakaryopoiesis-stimulating protein,** from Amgen Europe BV, for treatment idiopathic thrombocytopenic purpura

UPCOMING MEETINGS FOLLOWING THE DECEMBER 2008 COMP PLENARY MEETING

- 9th Workshop of the Eurordis Round Table of Companies on "Significant Benefit of Orphan Drugs: Impact on Clinical Development and Assessment" to be held on 12 December 2008 in Paris
- The ninety-seventh meeting of the COMP will be held on 7-8 January 2009.

[†] 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).

© EMEA 2008

OTHER MATTERS

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

- The appointment of Dr M. Todorova as the new Bulgarian COMP member.
- Discussion on the upcoming topics for the Informal COMP Meeting to be held on 9-10 March 2009 in Prague.
- Discussion on the Rare Diseases Task Force meeting held on 13 November 2008 and the publication of the Communication from the Commission to the European Parliament, the Council, the European Economic and Social Committee and the Committee of the Regions on Rare Diseases: Europe's challenges.
- Discussion on the 9th EPPOSI Workshop on Partnering for Rare Disease Therapy Development Sharing strategies and tools for access to diagnosis and treatment held on 16-17 October 2008 in Paris.
- Three 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

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

© EMEA 2008

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	116‡	86	31	-	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

‡ Data not complete for 2008 as there is still a deadline for submission in 2008.

© EMEA 2008

MEDICINAL PRODUCTS GRANTED A COMMUNITY DESIGNATION AS ORPHAN MEDICINAL PRODUCT SINCE THE NOVEMBER 2008 COMP PLENARY REPORT BY THE EUROPEAN COMMISSION

Active substance	2-[[3-({4-[(5-{2-[(3-Fluorophenyl)amino]-2-oxoethyl}-1H-pyrazol-3-yl)amino]-quinazolin-7-yl}oxy)propyl](ethyl)amino]ethyl dihydrogen phosphate trihydrate
Sponsor	Astra Zeneca AB
Orphan Indication	Treatment of acute myeloid leukaemia
COMP Opinion date	08/10/2008
Orphan Designation date	05/12/2008

Active substance	5-(ethylsulfonyl)-2-(naphthalen-2-yl)benzo[d]oxazole
Sponsor	Summit Oxford Limited
Orphan Indication	Treatment of Duchenne muscular dystrophy
COMP Opinion date	08/10/2008
Orphan Designation date	04/12/2008

Active substance	(R)-3-(4-(7H-pyrrolo[2,3-d]pyrimidin-4-yl)-1H-pyrazol-1-yl)-3-cyclopentylpropanenitrile phosphate
Sponsor	Incyte Corporation Ltd
Orphan Indication	Treatment of chronic idiopathic myelofibrosis
COMP Opinion date	10/09/2008
Orphan Designation date	07/11/2008

Active substance	Adeno-associated viral vector containing the human alpha-sarcoglycan gene
Sponsor	Généthon
Orphan Indication	Treatment of alpha-sarcoglycanopathy
COMP Opinion date	10/09/2008
Orphan Designation date	07/11/2008

Active substance	Autologous urothelial and smooth muscle cells
Sponsor	Choice Pharma Limited
Orphan Indication	Treatment of spinal cord injury
COMP Opinion date	10/09/2008
Orphan Designation date	07/11/2008

© EMEA 2008

Active substance	Carglumic acid
Sponsor	Orphan Europe SARL
Orphan Indication	Treatment of isovaleric acidaemia
COMP Opinion date	10/09/2008
Orphan Designation date	07/11/2008

Active substance	Carglumic acid
Sponsor	Orphan Europe SARL
Orphan Indication	Treatment of methylmalonic acidaemia
COMP Opinion date	10/09/2008
Orphan Designation date	07/11/2008

Active substance	Carglumic acid
Sponsor	Orphan Europe SARL
Orphan Indication	Treatment of propionic acidaemia
COMP Opinion date	10/09/2008
Orphan Designation date	07/11/2008

Active substance	Cenersen
Sponsor	EleosInc Limited
Orphan Indication	Treatment of chronic lymphocytic leukaemia
COMP Opinion date	08/10/2008
Orphan Designation date	03/12/2008

Active substance	Cysteamine hydrochloride
Sponsor	Orphan Europe SARL
Orphan Indication	Treatment of chronic lymphocytic leukaemia
COMP Opinion date	10/09/2008
Orphan Designation date	07/11/2008

Active substance	Daunorubicin (liposomal)
Sponsor	Diatos S.A.
Orphan Indication	Treatment of acute myeloid leukaemia
COMP Opinion date	08/10/2008
Orphan Designation date	03/12/2008

© EMEA 2008

Active substance	Ex-vivo expanded autologous human corneal epithelium containing stem cells
Sponsor	Chiesi Farmaceutici S.P.A.
Orphan Indication	Treatment of corneal lesions, with associated corneal (limbal) stem cell deficiency, due to ocular burns
COMP Opinion date	10/09/2008
Orphan Designation date	07/11/2008

Active substance	Filgrastim
Sponsor	Sygnis Bioscience GmbH& Co.KG
Orphan Indication	Treatment of spinal cord injury
COMP Opinion date	10/09/2008
Orphan Designation date	07/11/2008

Active substance	Gadodiamide (liposomal)
Sponsor	Dr Matthias Luz
Orphan Indication	Treatment of glioma
COMP Opinion date	08/10/2008
Orphan Designation date	03/12/2008

Active substance	Murine anti-CD22 antibody variable region fused to truncated Pseudomonas exotoxin 38
Sponsor	MEDIMMUNE Limited
Orphan Indication	Treatment of hairy cell leukaemia
COMP Opinion date	08/10/2008
Orphan Designation date	04/12/2008

Active substance	N2'-Deacetyl-N2'-[4-methyl-4-(oxobuthyldithio)-1-oxopentyl]-maytansine-chimerized anti-CD138 IgG4 monoclonal antibody
Sponsor	Biotest AG
Orphan Indication	Treatment of multiple myeloma
COMP Opinion date	08/10/2008
Orphan Designation date	03/12/2008

Active substance	Ofatumumab
Sponsor	Glaxo Group limited
Orphan Indication	Treatment of chronic lymphocytic leukaemia
COMP Opinion date	10/09/2008
Orphan Designation date	07/11/2008

© EMEA 2008

Active substance	Palifosfamide
Sponsor	Ziopharm Oncology Limited
Orphan Indication	Treatment of soft tissue sarcoma
COMP Opinion date	08/10/2008
Orphan Designation date	03/12/2008

Active substance	Recombinant human ADAMTS-13
Sponsor	Baxter AG
Orphan Indication	Treatment of thrombocytopenic purpura
COMP Opinion date	08/10/2008
Orphan Designation date	03/12/2008

Active substance	Recombinant human heparan-N-sulfatase
Sponsor	Shire Pharmaceutical Development limited
Orphan Indication	Treatment of mucopolysaccharidosis
COMP Opinion date	10/09/2008
Orphan Designation date	07/11/2008

Active substance	Recombinant human tissue non-specific alkaline phosphatase - Fc - deca-aspartate fusion protein
Sponsor	Europa Rx limited
Orphan Indication	Treatment of hypophosphatasia
COMP Opinion date	08/10/2008
Orphan Designation date	03/12/2008

Active substance	RNA, [P-deoxy-P-(dimethylamino)] (2',3'-dideoxy-2',3'-imino-2',3'-seco) (2'a→5') (C-m5U-C-C-A-A-C-A-m5U-C-A-A-G-G-A-A-G-A-m^5U-G-G-C-A-m^5U-m^5U-m^5U-C-m^5U-A-G), P-[4-[[2-[2-(2-hydroxyethoxy)ethoxy]ethoxy]carbonyl]-1-piperazinyl] N,N-dimethylaminophosphonamid
Sponsor	AVI BioPharma International Ltd
Orphan Indication	Treatment of Duchenne muscular dystrophy
COMP Opinion date	08/10/2008
Orphan Designation date	03/12/2008

Active substance	Yttrium (90Y) edotreotide
Sponsor	Molecular Insight Pharmaceuticals GmbH

© EMEA 2008

Orphan Indication	Treatment of gastro-entero-pancreatic neuroendocrine tumours
COMP Opinion date	08/10/2008
Orphan Designation date	04/12/2008

© EMEA 2008

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

Active substance	Invented name	Sponsor/applicant	EU Designation Number	Designated Orphan Indication
Adenovirus mediated *Herpes simplex* virus thymidine kinase (HKSV-tk) gene	Cerepro	Ark Therapeutics Ltd	EU/3/01/083	Treatment of high grade glioma with subsequent use of ganciclovir sodium

© EMEA 2008