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

6 August 2007
EMEA/COMP/328111/2007 Corr.

PRESS RELEASE
Committee for Orphan Medicinal Products
July 2007 Meeting

The eighty-first meeting of the Committee for Orphan Medicinal Products (COMP) took place on 24-25 July 2007. The Committee thanked warmly Dr Julia Dunne (CHMP representative), who will leave the COMP, for her successful contribution to the work of the Committee.

COMP Opinions for Orphan Medicinal Product Designation

The Committee adopted 12* positive opinions on orphan medicinal product designation during this meeting:

- 4-Amino-1-[5-O-[(2R,4S)-2-oxido-4-(4-pyridinyl)-1,3,2-dioxaphosphorinan-2-yl]-β-D-arabinofuranosyl]-2(1H)-pyrimidinone from Interface International Consultancy Ltd, **for treatment of hepatocellular carcinoma** (review time: day 41)
- 5'-O-(trans-9"-octadecenoyl)-1-ß-D-arabinofuranosyl cytosine from Clavis Pharma ASA, **for treatment of acute myeloid leukaemia** (review time: day 41)
- R-salbutamol sulphate from Astion Pharma A/S, **for treatment of cutaneous forms of lupus erythematosus** (review time: day 90)
- Cyclo {{(E,Z)-(2S,3R,4R)-3-hydroxy-4-methyl-2-(methylamino)nona-6,8-dienoyl}-L-2-aminobutyryl-N-methyl-glycyl-N-methyl-L-leucyl-L-valyl-N-methyl-L-leucyl-L-alanyl-D-alanyl-N-methyl-L-leucyl-N-methyl-L-leucyl-N-methyl-L-valyl} from Lux Biosciences GmbH, **for treatment of chronic non-infectious uveitis** (review time: day 90)
- Alpha-1 proteinase inhibitor (inhalation use) from CSL Behring GmbH, **for treatment of cystic fibrosis**, (review time: day 41)
- Alginate oligosaccharide (G-block) fragment from AlgiPharma AS, **for treatment of cystic fibrosis** (review time: day 90)
- Human coagulation factor X from Bio Products Laboratory, **for treatment of hereditary factor X deficiency**, (review time: day 41)
- Human heterologous liver cells (for infusion) from Cytonet GmbH & Co. KG, **for treatment of ornithine-transcarbamylase deficiency** (review time: day 41)
- N-(2-amino-phenyl)-4-[(4-pyridin-3-yl-pyrimidin-2-ylamino)-methyl] benzamide from Pharmion Ltd, **for treatment of Hodgkin's lymphoma** (review time: day 41)
- N-adamantanyl-N'-Geranyl-ethylenediamine from RLM Consulting, **for treatment of tuberculosis** (review time: day 41)

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* Amended number of adopted positive opinions (Human autologous bone-forming cells derived from bone marrow stem cells from Bone Therapeutics SA, for treatment of aseptic non-traumatic osteonecrosis was deleted from the list as the application will be discussed further at the September 2007 COMP meeting).

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- Naptumomab estafenatox from Active Biotech Research AB, **for treatment of renal cell carcinoma** (review time: day 41)
- Sulfonated monophosphorylated mannose oligosaccharide from Constella Group Ltd, **for treatment of hepatocellullar carcinoma** (review time: day 41)

Withdrawals of Orphan Medicinal Product Applications

The COMP noted that three applications for orphan medicinal product designation were withdrawn during evaluation.

Overview of orphan designation procedures

The European Commission granted 7 positive decisions on orphan designation[1] since the last COMP meeting on 26-27 June 2007 (see Annex 1).

The status of orphan designation procedures, to date for 2007, is summarised below:

Applications submitted	*Positive COMP Opinions*	*Applications withdrawn*	*Appeals ongoing*	*Final negative COMP Opinions*	*Designations granted by Commission*
77	51**	13	-	-	35

An overview of orphan designation procedures for 2000-2006 is provided in Annex 2.

Further information on designated orphan medicinal products is publicly available in the form of summarised COMP Opinions[2], which the Agency routinely publishes following adoption of the respective decisions on orphan designation by the European Commission.

Date of next COMP meeting

The next COMP meeting will be held on 11-12 September 2007.

NOTE: This Press Release, together with other information about the work of the EMEA, 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] These documents are available on the EMEA web-site

** Amended number of COMP positive opinions

Orphan Medicinal Product Designations received since the June 2007 COMP Meeting

Active substance	1-{3-[3-(4-chlorophenyl)propoxy]propyl}piperidine, hydrochloride
Sponsor	Bioprojet
Orphan Indication	Treatment of narcolepsy
Opinion receipt date	15 June 2007
Date of Commission Decision	10 July 2007

Active substance	Everolimus
Sponsor	Novartis Europharm Limited
Orphan Indication	Treatment of renal cell carcinoma
Opinion receipt date	4 May 2007
Date of Commission Decision	5 June 2007

Active substance	Fampridine
Sponsor	Dr Ulrich Granzer
Orphan Indication	Treatment of Guillain-Barré syndrome
Opinion receipt date	15 June 2007
Date of Commission Decision	10 July 2007

Active substance	Lusupultide
Sponsor	ALTANA Pharma AG
Orphan Indication	Treatment of aspiration pneumonitis requiring intubation and mechanical ventilation
Opinion receipt date	15 June 2007
Date of Commission Decision	10 July 2007

Active substance	Recombinant adeno-associated viral vector containing acid alpha-glucosidase-gene
Sponsor	The Matthews Consultancy Ltd
Orphan Indication	Treatment of glycogen storage disease type II (Pompe's disease)
Opinion receipt date	15 June 2007
Date of Commission Decision	9 July 2007

Active substance	Rilonacept
Sponsor	Regeneron UK Limited
Orphan Indication	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)
Opinion receipt date	15 June 2007
Date of Commission Decision	10 July 2007

Active substance	Talactoferrinum alfa
Sponsor	Agennix Limited
Orphan Indication	Treatment of renal cell carcinoma
Opinion receipt date	4 May 2007
Date of Commission Decision	5 June 2007

Overview of Procedures for Orphan Medicinal Product Designation for 2000-2006

Year	*Applications submitted*	*Positive COMP Opinions*	*Applications withdrawn*	*Final negative COMP Opinions*	*Designations granted by Commission*
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