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COMMITTEE FOR ORPHAN MEDICINAL PRODUCTS
OCTOBER 2007 PLENARY MEETING
MONTHLY REPORT

The Committee for Orphan Medicinal Products (COMP) held its eighty-third plenary meeting on 9-10 October 2007.

ORPHAN MEDICINAL PRODUCT DESIGNATION

The COMP adopted 16 positive opinions recommending the following medicinal products for designation as orphan medicinal products to the European Commission:

- **(1R,2R)-octanoic acid[2-(2',3'-dihydro-benzo[1,4] dioxin-6'-yl)-2-hydroxy-1-pyrrolidin-1-ylmethyl-ethyl]-amide-L-tartaric acid salt** from Genzyme Europe BV for treatment of Gaucher disease (review date: day 90)
- **(S)-2-nitro-6-(4-trifluoromethoxy)benzyloxy)-6,7-dihydro-5H-imidazo[2,1-b] [1,3] oxazine** from Dr Ulrich Granzer for treatment of tuberculosis (review date: day 59)
- **17-(allylamino)-17-demethoxygeldanamycin, hydroquinone, hydrochloride** from MedImmune Oncology, Inc. for treatment of malignant gastrointestinal stromal tumours (review date: day 59)
- **3-methoxy-pregnelonone** from MAPREG SAS for treatment of spinal cord injury (review date: day 59)
- **Artesunate** from Sigma-tau Pharma UK for treatment of malaria (review date: day 59)
- **Azacitidine** from Pharmion Ltd for treatment of acute myeloid leukaemia (review date: day 59)
- **Chimeric-anti-interleukin-6 monoclonal antibody** from Centocor, B.V. for treatment of Castleman's disease (review date: day 59)
- **Doxorubicin hydrochloride (drug eluting beads)** from CellMed AG for treatment of glioma (review date: day 59)
- **Heterologous human adult liver derived stem cells** from Prof. Etienne Sokal for treatment of Crigler-Najjar syndrome (review date: day 59)
- **Interferon beta** from Faron Pharmaceuticals Limited for treatment of acute lung injury (review date: day 59)
- **Irinotecan hydrochloride (drug eluting beads)** from CellMed AG for treatment of glioma (review date: day 59)
- **Olaparib** from AstraZeneca AB for treatment of ovarian cancer (review date: day 59)
- **Picoplatin** from Kendle International Ltd for treatment of small cell lung cancer (review date: day 59)

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- **Recombinant human hepatitis C monoclonal antibody against C4 region of E1** from GENimmune N.V. for prevention of recurrent hepatitis C virus induced liver disease in liver transplant recipients (review date: day 90)
- **Recombinant human rod-derived cone viability factor** from Fovea Pharmaceuticals SA for treatment of retinitis pigmentosa (review date: day 59)
- **Terguride** from Ergonex Licensing and Regulatory Services AG c/o Terra Sana Truhand und Verwaltung AG for treatment of pulmonary arterial hypertension (review date: day 59)

Negative opinion

The COMP recommends that its previous negative opinion should not be revised and recommends the refusal of the orphan medicinal product designation for the below-mentioned medicinal product. This follows the intent to appeal against the negative opinion by the sponsor.

- **Chelidonii radix special liquid extract**, Now Pharm AG, 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.

OTHER INFORMATION ON THE ORPHAN MEDICINAL PRODUCT DESIGNATION

Lists of questions

The COMP adopted six 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

Two oral hearings took place.

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 the 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 via written procedure on 30 July 2007. The opinion recommends the European Commission the maintenance of the following orphan medicinal products in the Community registry:

- **Yondelis (ecteinascidin 743)** from PharmaMar SA Sociedad Unipersonal, for treatment of soft tissue sarcoma

[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)

UPCOMING MEETINGS FOLLOWING THE OCTOBER 2007 COMP PLENARY MEETING

- The eighty-fourth meeting of the COMP will be held on 7-8 November 2007.
- The Informal COMP meeting will be held on 24-26 October 2007 in Lisbon, Portugal.

ORGANISATIONAL MATTERS

The main organisational topics during the October 2007 COMP meeting were related to:

- The appointment of Dr Vessela Boudinova as the new Bulgarian member of the COMP.
- Four Protocol Assistance letters were adopted.
- Discussion on the Communication from the Commission regarding European Action in the Field of rare Diseases.
- Outcome on the DG Research Conference Rare Disease Research held on 13 September 2007 in Brussels, Belgium.
- Outcome of the COMP Working Group with Interested Parties meeting held on 26 September 2007.
- Discussion on the Eurordis Survey on Orphan Drug availability in Europe

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
2007	100	85	15	1	58
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 OCTOBER 2007 COMP PLENARY REPORT BY THE EUROPEAN COMMISSION

Active substance	4-Amino-1-[5-O-[(2*R*,4S)-2-oxido-4-(4-pyridinyl)-1,3,2-dioxaphosphorinan-2-yl]-β-D-arabinofuranosyl]-2(1H)-pyrimidinone
Sponsor	Interface International Consultancy Ltd
Orphan Indication	Treatment of hepatocellular carcinoma
COMP Opinion date	25/07/2007
Orphan Designation date	14/09/2007

Active substance	5'-O-(trans-9"-octadecenoyl)-1-ß-D-arabinofuranosyl cytosine
Sponsor	Clavis Pharma ASA
Orphan Indication	Treatment of acute myeloid leukaemia
COMP Opinion date	25/07/2007
Orphan Designation date	14/09/2007

Active substance	Alginate oligosaccharide (G-block) fragment
Sponsor	AlgiPharma AS
Orphan Indication	Treatment of cystic fibrosis
COMP Opinion date	25/07/2007
Orphan Designation date	14/09/2007

Active substance	Alpha-1 proteinase inhibitor (inhalation use)
Sponsor	CSL Behring GmbH
Orphan Indication	Treatment of cystic fibrosis
COMP Opinion date	25/07/2007
Orphan Designation date	14/09/2007

Active substance	Aviptadil
Sponsor	mondoBIOTECH Laboratories Anstalt
Orphan Indication	Treatment of sarcoidosis
COMP Opinion date	30/07/2007
Orphan Designation date	14/09/2007

Active substance	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}
Sponsor	Lux Biosciences GmbH
Orphan Indication	Treatment of chronic non-infectious uveitis
COMP Opinion date	25/07/2007
Orphan Designation date	14/09/2007

Active substance	Human coagulation factor X
Sponsor	Bio Products Laboratory
Orphan Indication	Treatment of hereditary factor X deficiency
COMP Opinion date	25/07/2007
Orphan Designation date	17/09/2007

Active substance	Human heterologous liver cells (for infusion)
Sponsor	Cytonet GmbH & Co. KG
Orphan Indication	Treatment of ornithine-transcarbamylase deficiency
COMP Opinion date	25/07/2007
Orphan Designation date	14/09/2007

Active substance	N-(2-amino-phenyl)-4-[(4-pyridin-3-yl-pyrimidin-2-ylamino)-methyl] benzamide
Sponsor	Pharmion Ltd
Orphan Indication	Treatment of Hodgkin's lymphoma
COMP Opinion date	25/07/2007
Orphan Designation date	14/09/2007

Active substance	N-adamantanyl-N'-geranyl-ethylenediamine
Sponsor	RLM Consulting
Orphan Indication	Treatment of tuberculosis
COMP Opinion date	25/07/2007
Orphan Designation date	14/09/2007

Active substance	Naptumomab estafenatox
Sponsor	Active Biotech Research AB
Orphan Indication	Treatment of renal cell carcinoma
COMP Opinion date	25/07/2007
Orphan Designation date	14/09/2007

Active substance	R-salbutamol sulphate
Sponsor	Astion Pharma A/S
Orphan Indication	Treatment of cutaneus forms of lupus erythromatosus
COMP Opinion date	25/07/2007
Orphan Designation date	14/09/2007

Active substance	Sulfonated monophosphorylated mannose oligosaccharide
Sponsor	Constella Group Ltd
Orphan Indication	Treatment of hepatocellullar carcinoma
COMP Opinion date	25/07/2007
Orphan Designation date	14/09/2007