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

21 July 2005
EMEA/COMP/233637/2005

PRESS RELEASE
Committee for Orphan Medicinal Products
July 2005 Meeting

The fifty-ninth meeting of the Committee for Orphan Medicinal Products (COMP) took place on 12-13 July 2005.

The Committee welcomed Prof. B. Blöchl-Daum who will replace Prof. B. Jilma as the Austrian COMP member. The Committee thanked Prof. Jilma for his contribution and collaboration.

COMP Opinions

The Committee adopted 17 positive opinions on orphan medicinal product designation during this meeting:

- (1R,2R,4S)-4-{(2R)-2-[(3S,6R,7E,9R,10R,12R,14S,15E, 17E,19E,21S,23S,26R,27R,34aS)-9,27-dihydroxy-10,21-dimethoxy-6,8,12,14,20,26-hexamethyl-1,5,11,28,29-pentaoxo-1,4,5,6,9,10,11,12,13,14,21,22,23,24,25,26,27,28,29,31,32,33,34,34a-tetra-cosahydro-3H-23,27-epoxypyrido[2,1-c][1,4]oxazacyclohentriacontin-3-yl]propyl}-2-methoxy-cyclohexyldimethyl-phosphinate, from Orphix Consulting GmbH, for treatment of **soft tissue sarcoma** (review time: 32 days).

- (1R,2S) 6-bromo-alpha-[2-(dimethylamino)ethyl]-2-methoxy-alpha-(1-naphthyl)-beta-phenyl-3-quinolineethanol, from Tibotec Pharmaceuticals Ltd., for treatment of **tuberculosis** (review time: 88 days).

- 2-{4-[(5,6-diphenylpyrazin-2-yl)(isopropyl)amino]butoxy}-N-(methylsulfonyl)acetamide, from Nippon Shinyako Co. Ltd, for treatment of **pulmonary arterial hypertension and chronic thromoembolic pulmonary hypertension** (review time: 32 days).

- (2S)-2-[(4R)-2-oxo-4-propyltetrahydro-1H-pyrrol-1-yl] butanamide, from UCB SA, for treatment of **progressive myoclonic epilepsies** (review time: 32 days).

- A mixture of anti-CD3 mAb (SPV-T3a)-ricin A chain fusion protein and anti-CD7 mAb (WT1)-ricin A chain fusion protein, from Henogen SA, for treatment of **graft-versus-host-disease** (review time: 32 days).

- Autologous CD34+ cells transfected with retroviral vector containing adenosine deaminase gene, from Fondazione Telethon, for treatment of **severe combined immunodeficiency (SCID) due to adenosine deaminase (ADA) deficiency** (review time: 32 days).

- Chimeric monoclonal antibody to shiga-toxin 1 and 2, from Albany Regulatory Consulting Ltd, for treatment of **shiga-toxin producing bacterial infection** (review time: 32 days).

- Extract of *Sorghum bicolour* leaf, *Pterocarpus osun* stem, *Piper guineense* seed and *Caryophylli* flower, from Xechem UK Ltd., for treatment of **sickle cell disease** (review time: 88 days).

- Human autologous mesenchymal adult stem cells extracted from adipose tissue, from CELLERIX S.L., for treatment of **anal fistula** (review time: 88 days).

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.eu.int http://www.emea.eu.int

- Imatinib mesilate, from Novartis Europharm Limited, for treatment of **dermatofibrosarcoma protuberans** (review time: 32 days).
- Imatinib mesilate, from Novartis Europharm Limited, for treatment of **acute lymphoblastic leukaemia** (review time: 32 days).
- Imatinib mesilate, from Novartis Europharm Limited, for treatment of **mastocytosis** (review time: 32 days).
- Mecasermin, from MDS Pharma Services GB Limited, for treatment of **primary growth hormone insensitivity syndrome** (review time: 88 days).
- Sapropterin, from Dr Gunter Schaub, for treatment of **hyperphenylalaninemia** (review time: 32 days).
- Sodium valproate, from the Jennifer Trust for Spinal Muscular Atrophy, for treatment of **5q spinal muscular atrophy** (review time: 32 days).
- Treprostinil diethanolamine (oral use), from United Therapeutics Europe Ltd, for treatment of **pulmonary arterial hypertension and chronic thromboembolic pulmonary hypertension** (review time: 32 days).
- Troxacitabine, from GMG BioBusiness, for treatment of **acute myeloid leukaemia** (review time: 32 days).

Three oral explanations took place during the meeting. The COMP noted that four applications for orphan medicinal product designation were withdrawn by sponsors.

Overview of orphan designation procedures

The European Commission granted ten positive decisions on orphan designation[1] since the last COMP meeting on 14-15 June 2005 (see Annex 1).

The status of orphan designation procedures, to date for 2005, is summarised below:

Applications submitted	*Positive COMP Opinions*	*Applications withdrawn*	*Appeals ongoing*	*Final negative COMP Opinions*	*Designations granted by Commission*
62	57	15	-	-	40

Three negative decisions on orphan medicinal product designation were also adopted by the European Commission in June-July 2005.

On 16 June 2005, a negative decision for a designation application that concerned a sub-set of Peyronie's disease was adopted on the grounds of prevalence. The sponsor had not provided adequate justification for limiting the proposed orphan indication to the active phase of Peyronie's disease and had not established that the proposed indication affected not more than 5 in 10,000 persons in the Community at the time the application was made.

On 24 June 2005 and 7 July 2005, two negative decisions for designation applications proposed for patent ductus arteriosus (prevention and treatment) were adopted. In both applications, the sponsor had failed to provide adequate justification that the EU prevalence of the proposed target population laid below the 5 in 10,000 threshold for designation. In addition for the treatment application, satisfactory methods of treatment of the condition were deemed to be available in the Community, and adequate justification of significant benefit to those affected by the condition was not provided.

[1] Details of all orphan designations granted to date by the European Commission are entered in the Community Register of Orphan Medicinal Products (http://pharmacos.eudra.org/F2/register/index.htm)

An overview of orphan designation procedures for 2000-2004 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 8-9 September 2005.

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.eu.int/

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

[2] These documents are available on the EMEA web-site.

Medicinal products Designated as Orphan Medicinal Products since the June 2005 COMP Meeting

Active substance	Pegylated arginine deiminase
Sponsor	Dr. Francesco Izzo
Orphan Indication	Treatment of hepatocellular carcinoma
Opinion receipt date	23/05/2005
Date of Commission Decision	20/06/2005

Active substance	H-D-Asp-D-Gln-D-Ser-D-Arg-D-Pro-D-Val-DGln-D-Pro-D-Phe-D-Leu-D-Asn-D-Leu-D-Thr-D-Thr-D-Pro-D-Arg-D-Lys-D-Pro-D-Arg-D-Pro-D-Pro-D-Arg-D-Arg-D-Arg-D-Gln-D-Arg-D-Arg-D-Lys-D-Lys-DArg-D-Gly-NH2
Sponsor	Laboratoire Auris SAS
Orphan Indication	Treatment of acute sensorineural hearing loss (acute acoustic trauma, sudden deafness and surgery induced acoustic trauma)
Opinion receipt date	23/05/2005
Date of Commission Decision	16/06/2005

Active substance	Nelarabine
Sponsor	GlaxoSmithKline Research & Development Limited
Orphan Indication	Treatment of acute lymphoblastic leukaemia
Opinion receipt date	23/05/2005
Date of Commission Decision	16/06/2005

Active substance	Human monoclonal antibody against HLA-DR
Sponsor	GPC Biotech AG
Orphan Indication	Treatment of Hodgkin's lymphoma
Opinion receipt date	23/05/2005
Date of Commission Decision	16/06/2005

Active substance	4-[3-(methylsulfonyl)phenyl]-1-propylpiperidine x HC1
Sponsor	A Carlsson Research AB
Orphan Indication	Treatment of Huntington's disease
Opinion receipt date	23/05/2005
Date of Commission Decision	20/06/2005

Active substance	Bovine bile extract
Sponsor	GMG BioBusiness Ltd.
Orphan Indication	Treatment of pancreatic cancer
Opinion receipt date	23/05/2005
Date of Commission Decision	20/06/2005

Active substance	N-(methyl-diazacyclohexyl-methylbenzamide)-azaphenyl-aminothiopyrrole
Sponsor	AB Science
Orphan Indication	Treatment of multiple myeloma
Opinion receipt date	23/05/2005
Date of Commission Decision	20/06/2005

Active substance	Soluble yeast beta-1,3/1,6-glucan
Sponsor	Biotec Pharmacon ASA
Orphan Indication	Prevention of oral mucositis in head and neck cancer patients undergoing radiation therapy
Opinion receipt date	23/05/2005
Date of Commission Decision	16/06/2005

Active substance	Humanised antibody fragment (Ep-CAM)-truncated Pseudomonas exotoxin A fusion protein
Sponsor	CanReg (Europe) Limited
Orphan Indication	Treatment of Ep-CAM-positive squamous cell carcinoma of the head and neck
Opinion receipt date	23/05/2005
Date of Commission Decision	20/06/2005

Active substance	Hepatitis C Immunoglobulin
Sponsor	Nabi Biopharmaceuticals Europe Ltd
Orphan Indication	Prevention of recurrent hepatitis C virus induced liver disease in liver transplant recipients
Opinion receipt date	31/05/2005
Date of Commission Decision	08/07/2005

Overview of Procedures for Orphan Medicinal Product Designation for 2000-2004

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