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

18 May 2005
EMEA/COMP/162001/2005

PRESS RELEASE
Committee for Orphan Medicinal Products
May 2005 Meeting

The fifty-seventh meeting of the Committee for Orphan Medicinal Products (COMP) took place on 11-12 May 2005.

The Committee welcomed Prof. Fernando de Andrés -Trelles who will replace Dr. José Félix Olalla Marañón as the Spanish COMP member. The Committee thanked Dr. Olalla Marañón for his contribution and collaboration.

Prof. Bert Leufkens and Dr. Sonja van Weely, from the Netherlands Steering Committee on Orphan Drugs, attended the meeting to present the Rare (Orphan) Diseases chapter of the WHO Report 'Priority Medicines for Europe and the World' to the Committee.

COMP Opinions

The Committee adopted 10 positive opinions on orphan medicinal product designation during this meeting:

- 4-[3-(methylsulfonyl)phenyl]-1-propylpiperidine x HC1, from A Carlsson Research AB, for treatment of **Huntington's disease** (review time: 53 days).
- Bovine bile extract, from GMG BioBusiness Ltd, for treatment of **pancreatic cancer** (review time: 88 days).
- H-D-Asp-D-Gln-D-Ser-D-Arg-D-Pro-D-Val-D-Gln-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-D-Arg-D-Gly-NH2, from Laboratoires Auris SAS, for treatment of **acute sensorineural hearing loss (acute acoustic trauma, sudden deafness and surgery induced acoustic trauma)** (review time: 53 days).
- Hepatitis C Immunoglobulin, from Nabi Biopharmaceuticals Europe Ltd, for prevention of **recurrent hepatitis C virus induced liver disease in liver transplant recipients** (review time: 88 days).
- Human monoclonal antibody against HLA-DR, from GPC Biotech AG, for treatment of **Hodgkin's lymphoma** (review time: 53 days).
- Humanised antibody fragment (Ep-CAM)-truncated Pseudomonas exotoxin A fusion protein, from CanReg (Europe) Limited, for treatment of **Ep-CAM-positive squamous cell carcinoma of the head and neck** (review time: 88 days).
- N-(methyl-diazacyclohexyl-methylbenzamide)-azaphenyl-aminothiopyrrole, from AB Science, for treatment of **multiple myeloma** (review time: 53 days).
- Nelarabine, from GlaxoSmithKline Research & Development Limited, for treatment of **acute lymphoblastic leukaemia** (review time: 53 days).
- Pegylated arginine deiminase, from Dr. Francesco Izzo, for treatment of **hepatocellular carcinoma** (review time: 53 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

- Soluble yeast beta-1,3/1,6- glucan, from Biotec Pharmacon ASA, for prevention of **oral mucositis in head and neck cancer patients undergoing radiation therapy** (review time: 88 days).

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

Overview of orphan designation procedures

The European Commission granted seven positive decisions on orphan designation[1] since the last COMP meeting on 6-7 April 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*
30	35	10	-	-	21

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.

Applications for Marketing Authorisation for Orphan Medicinal Products

Details of those designated orphan medicinal products that have been the subject of a centralised application for marketing authorisation since the last COMP meeting are provided in Annex 3.

Date of next COMP meeting

The next COMP meeting will be held on 14-15 June 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

[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)

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

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

Active substance	Melatonin
Sponsor	ICON Consulting
Orphan Indication	Treatment of Non-24-Hour Sleep-Wake Disorders in blind people with no light perception
Opinion receipt date	15/03/2005
Date of Commission Decision	11/04/2005

Active substance	Paromomycin sulphate
Sponsor	OneWorld Health
Orphan Indication	Treatment of visceral leishmaniasis
Opinion receipt date	15/03/2005
Date of Commission Decision	11/04/2005

Active substance	Ambrisentan
Sponsor	Uppsala Medical Information System AB
Orphan Indication	Treatment of pulmonary arterial hypertension and chronic thromboembolic pulmonary hypertension
Opinion receipt date	15/03/2005
Date of Commission Decision	11/04/2005

Active substance	Autologous Tumor-Derived gp96 Heat Shock Protein-Peptide Complex
Sponsor	Antigenics Therapeutics Limited
Orphan Indication	Treatment of renal cell carcinoma
Opinion receipt date	15/03/2005
Date of Commission Decision	11/04/2005

Active substance	Estradiol Hemihydrate and Progesterone
Sponsor	B. Braun Melsungen AG
Orphan Indication	Prevention of bronchopulmonary dysplasia in premature neonates of less than 30 weeks of gestational age
Opinion receipt date	15/03/2005
Date of Commission Decision	11/04/2005

Active substance	Histamine dihydrochloride
Sponsor	Maxim Pharmaceuticals Europe Ltd
Orphan Indication	Treatment of acute myeloid leukaemia
Opinion receipt date	15/03/2005
Date of Commission Decision	11/04/2005

Active substance	Humanized Agonistic Anti-CD28 Monoclonal Antibody
Sponsor	TeGenero AG
Orphan Indication	Treatment of B-cell Chronic Lymphocytic Leukaemia
Opinion receipt date	15/03/2005
Date of Commission Decision	11/04/2005

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

Overview of Designated Orphan Medicinal Products that have been the subject of a Centralised Application for Marketing Authorisation

- update since the last COMP meeting on 6-7 April 2005 -

Active substance	***Sponsor/applicant***	***EU Designation Number & Date of Orphan Designation***	***Designated Orphan Indication***
Stiripentol (Diacomit)	Laboratoires BIOCODEX	EU/3/01/071 05/12/2001	Treatment of severe myoclonic epilepsy in infancy
4-(3,5-bis-(hydroxy-phenyl)-1,2,4) triazol-1-yl)-benzoic acid (Exjade)	Novartis Europharm Limited	EU/3/02/092 13/03/2002	Treatment of chronic iron overload requiring chelation therapy