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

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PRESS RELEASE
Committee for Orphan Medicinal Products
July 2004 Meeting

The forty-eighth meeting of the Committee for Orphan Medicinal Products (COMP) took place on 21-22 July 2004.

The Committee adopted 8 positive opinions on orphan medicinal product designation for:

- 5-10-Methylene-tetrahydrofolate, from Biofol AB, for treatment of pancreatic cancer in combination with 5-fluorouracil (review time: 39 days).
- Anti epidermal growth factor receptor antibody h-R3, from Oncoscience AG, for treatment of glioma (review time: 39 days).
- Heparin sodium, from Prof. Dr. W Seeger, for treatment of idiopathic pulmonary fibrosis (review time: 88 days).
- HLA-B27-derived peptide (amino acid 125-138), from Lynkeus BioTech GmbH, for treatment of autoimmune uveitis (review time: 39 days).
- Homoharringtonine, from Stragen France SAS, for treatment of chronic myeloid leukaemia (review time: 39 days).
- Pancreatic enzymes (cross linked enzyme crystal lipase, protease, amylase), from Dr Falk Pharma GmbH, for treatment of malabsorption due to exocrine pancreatic enzyme insufficiency (review time: 88 days).
- Recombinant human interleukin-21, from Novo Nordisk A/S, for treatment of renal cell carcinoma (review time: 39 days).
- Sodium dichloroacetate, from EBD Group, for treatment of systemic monochloroacetate poisoning (review time: 39 days).

No negative opinions on orphan medicinal product designation were adopted. One oral explanation took place during the meeting and resulted in a positive opinion. The COMP noted that one application for orphan medicinal product designation was withdrawn by a sponsor.

The European Commission granted six positive decisions on orphan designation[1] since the last COMP meeting on 15-16 June 2004 (see Annex 1).

The status of orphan designation procedures, to date for 2004, is summarised below:

Applications submitted	*Positive COMP Opinions*	*Applications withdrawn*	*Appeals ongoing*	*Final negative COMP Opinions*	*Designations granted by Commission*
51	41	10	-	-	24

An overview of orphan designation procedures for 2000-2003 is provided in Annex 2.

[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)

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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.

The Committee discussed and endorsed a revised version of the Commission guideline (ENTR/6283/00 Revision 2)[3] on the format and content of applications for designation as orphan medicinal products, which has been updated to incorporate comments received during the consultation period. Revision 2 of the guideline incorporates Icelandic and Norwegian participation in the designation process and makes reference to the Commission Communication (2003/C 178/02)[4]. In addition, some minor editorial changes have been implemented. In preparing an application for orphan medicinal product designation, sponsors are requested to follow this guideline.

The next COMP meeting will be held on 8-9 September 2004.

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/

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

3 ENTR/6283/00 is available on the EMEA and Commission (http://dg3.eudra.org) web-sites

4 Commission Communication is published in the Official Journal of the European Union (C 178, 29.07.2003, p. 2) and is available on the Commission web-site (http://pharmacos.eudra.org/F2/)

Medicinal products Designated as Orphan Medicinal Products since the June 2004 COMP Meeting

Active substance	Vascular endothelial growth factor-D gene in an adenoviral vector for use with a collagen collar
Sponsor	Ark Therapeutics Limited
Orphan Indication	Prevention of stenosis in synthetic grafts used in haemodialysis
Opinion receipt date	29/4/2004
Date of Commission Decision	8/6/2004

Active substance	Muramyl tripeptide phosphatidyl ethanolamine
Sponsor	Immuno-Designed Molecules, SA
Orphan Indication	Treatment of osteosarcoma
Opinion receipt date	25/5/2004
Date of Commission Decision	21/6/2004

Active substance	5'-CTG CCA CGT TCT CCT GC-(2' methoxy)A-(2' methoxy)C-(2' methoxy)C-3'
Sponsor	PPD Global Ltd
Orphan Indication	Treatment of myasthenia gravis
Opinion receipt date	25/5/2004
Date of Commission Decision	21/6/2004

Active substance	Suberoylanilide hydroxamic acid
Sponsor	Stringer Consultancy Services Ltd
Orphan Indication	Treatment of cutaneous T-cell lymphoma
Opinion receipt date	25/5/2004
Date of Commission Decision	21/6/2004

Active substance	HLA-A2 restricted CD8 T-cell line expressing MART-1 T-cell receptor
Sponsor	CellCure ApS
Orphan Indication	Treatment of MART-1 positive malignant melanoma in HLA-A2 positive patients
Opinion receipt date	25/5/2004
Date of Commission Decision	21/6/2004

Active substance	Aztreonam lysinate (inhalation use)
Sponsor	MoRa Pharm GmbH
Orphan Indication	Treatment of gram negative bacterial lung infection in cystic fibrosis
Opinion receipt date	25/5/2004
Date of Commission Decision	21/6/2004

Overview of Procedures for Orphan Medicinal Product Designation for 2000-2003

Year	*Applications submitted*	*Positive COMP Opinions*	*Applications withdrawn*	*Final negative COMP Opinions*	*Designations granted by Commission*
2003	87	54	41	1	55
2002	80	43	30	3	49
2001	83	64	27	1	64
2000	72	26	6	0	14