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

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PRESS RELEASE
Committee for Orphan Medicinal Products
September 2004 Meeting

The forty-ninth meeting of the Committee for Orphan Medicinal Products (COMP) took place on 8-9 September 2004.

COMP Opinions

The Committee adopted 14 positive opinions on orphan medicinal product designation for:

- l, 1'-[1,4-phenylenebis (methylene)]-bis-1,4,8,11- tetraazacyclotetradecane, from Orphix Consulting GmbH, for treatment to mobilize progenitor cells prior to **stem cell transplantation** (review time: 60 days).
- Adeno-associated viral vector containing the human gamma-sarcoglycan gene, from Généthon, for treatment of **gamma-sarcoglycanopathy** (review time: 60 days).
- Biotinylated anti-tenascin monoclonal antibody for use with 90-Yttrium, from Sigma-Tau Industrie Farmaceutiche Riunite S.p.A., for treatment of **glioma** (review time: 60 days).
- Deferoxamine mesilate, from Neuraxo Biotec GmbH, for treatment of **traumatic spinal cord injury** (review time: 60 days).
- Deuterium oxide, from BDD Berolina Drug Development GmbH, for treatment of **pancreatic cancer** (review time: 88 days).
- Dexamethasone sodium phosphate encapsulated in human erythrocytes, from Dideco S.p.A., for treatment of **cystic fibrosis** (review time: 60 days).
- Doxorubicine polyisohexylcyanoacrylate nanoparticles, from BioAlliance Pharma, for treatment of **hepatocellular carcinoma** (review time: 60 days).
- Homoharringtonine, from Stragen France SAS, for treatment of **acute myeloid leukaemia** (review time: 60 days).
- Recombinant human insulin-like growth factor-I/recombinant human insulin-like growth factor binding protein-3, from Dr. Geoffrey Allan, for treatment of **Leprechaunism** (review time: 88 days).
- Recombinant human insulin-like growth factor-I/recombinant human insulin-like growth factor binding protein-3, from Dr. Geoffrey Allan, for treatment of **Rabson-Mendenhall syndrome** (review time: 88 days).
- Recombinant human insulin-like growth factor-I/recombinant human insulin-like growth factor binding protein-3, from Dr. Geoffrey Allan, for treatment of **Type A extreme insulin resistance syndrome** (review time: 88 days).
- Recombinant human insulin-like growth factor-I/recombinant human insulin-like growth factor binding protein-3, from Dr. Geoffrey Allan, for treatment of **Type B extreme insulin resistance syndrome** (review time: 88 days).

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- Rufinamide, from Eisai Limited, for treatment of **Lennox-Gastaut syndrome** (review time: 60 days).

- Sitaxentan sodium, from PPD Global Ltd, for treatment of **pulmonary arterial hypertension and chronic thromboembolic pulmonary hypertension** (review time: 88 days).

No negative opinions on orphan medicinal product designation were adopted. Two oral explanations took place during the meeting, one resulting in a positive opinion and one in a withdrawal.

Commission Decisions on Designation

The European Commission granted twenty positive decisions on orphan designation[1] since the last COMP meeting on 21-22 July 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*
69	55	11	-	-	44

One negative decision on orphan medicinal product designation was also adopted by the European Commission on 24 August 2004. The grounds for the negative decision were based on the prevalence criterion. The sponsor had not established that the condition that was the subject of the designation application concerned, malignant melanoma, affected not more than 5 in 10,000 persons in the Community at the time the application was made and thus the designation criteria were not met.

An overview of orphan designation procedures for 2000-2003 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.

Draft Guidance on Medical Plausibility/Significant Benefit

The Committee finalised a draft Guideline on the 'Elements required to support the medical plausibility and the assumption of significant benefit for an orphan designation' (COMP/1527/03), which will be released on the EMEA web-site for a 6 months consultation period. This document, which supplements the general guidance contained in the Commission Guideline on the format and content of applications for designation as orphan medicinal products (ENTR/6283/00), reflects the experience that COMP has gained over recent years following the evaluation of several hundred applications for orphan medicinal product designation.

New Transparency Initiative for Orphan Medicinal Products

The Management Board of the EMEA has recently endorsed a new initiative to increase the transparency of orphan medicinal products in accordance with a recommendation contained within the 'Communication from the Commission on Regulation (EC) No 141/2000 of the European Parliament and of the Council on Orphan Medicinal Products'[3].

When a sponsor of a designated orphan medicinal product submits a centralised application for marketing authorisation to the EMEA, the following information will now be made public in the Press Release of the COMP and the monthly report of the CHMP:
- the active substance
- the sponsor/applicant
- the designated orphan indication[4]

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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://pharmacos.eudra.org/F2/register/index.htm)

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

3 OJ C 178, 29.07.2003, p.2

4 The proposed therapeutic indication as specified in section 4.1 of the Draft Summary of Product Characteristics will not be published at this stage, only the orphan indication as designated

In Annex 3 of this press release, this information is being published for the first time for those orphan medicinal products that have been the subject of a centralised application for marketing authorisation since July 2003, the date of publication of the Commission Communication. Future applications for marketing authorisation will be announced in the Committees' respective press release/monthly report and will be recorded in the Community Register of Orphan Medicinal Products[1].

Date of next COMP meeting

The next COMP meeting will be held on 6-7 October 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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Medicinal products Designated as Orphan Medicinal Products since the July 2004 COMP Meeting

Active substance	(R, S)-3-(bromomethyl)-3-butanol-1-yl-disphosphate
Sponsor	Innate Pharma
Orphan Indication	Treatment of renal cell carcinoma
Opinion receipt date	25 June 2004
Date of Commission Decision	29 July 2004

Active substance	Porfimer sodium (for use with photodynamic therapy)
Sponsor	Axcan Pharma International BV
Orphan Indication	Treatment of cholangiocarcinoma
Opinion receipt date	25 June 2004
Date of Commission Decision	29 July 2004

Active substance	Mepolizumab
Sponsor	SmithKline Beecham plc
Orphan Indication	Treatment of hypereosinophilic syndrome
Opinion receipt date	25 June 2004
Date of Commission Decision	29 July 2004

Active substance	Midostaurin
Sponsor	Novartis Europharm Limited
Orphan Indication	Treatment of acute myeloid leukaemia
Opinion receipt date	25 June 2004
Date of Commission Decision	29 July 2004

Active substance	Sinapultide, dipalmitoylphosphatidylcholine, palmitoyl-oleoyl phosphatidylglycerol and palmitic acid
Sponsor	GMG BioBusiness Ltd
Orphan Indication	Prevention of respiratory distress syndrome in premature neonates of less than 32 weeks of gestational age
Opinion receipt date	25 June 2004
Date of Commission Decision	29 July 2004

Active substance	Sinapultide, dipalmitoylphosphatidylcholine, palmitoyl-oleoyl phosphatidylglycerol and palmitic acid
Sponsor	GMG BioBusiness Ltd
Orphan Indication	Treatment of respiratory distress syndrome in premature neonates of less than 37 weeks of gestational age
Opinion receipt date	25 June 2004
Date of Commission Decision	29 July 2004

Active substance	Sorafenib tosylate
Sponsor	Bayer Healthcare AG
Orphan Indication	Treatment of renal cell carcinoma
Opinion receipt date	25 June 2004
Date of Commission Decision	29 July 2004

Active substance	Acetylsalicylic acid
Sponsor	Bayer Vital GmbH
Orphan Indication	Treatment of polycythemia vera
Opinion receipt date	25 June 2004
Date of Commission Decision	29 July 2004

Active substance	Defibrotide
Sponsor	Gentium S.p.A.
Orphan Indication	Prevention of hepatic veno-occlusive disease
Opinion receipt date	25 June 2004
Date of Commission Decision	29 July 2004

Active substance	Defibrotide
Sponsor	Gentium S.p.A.
Orphan Indication	Treatment of hepatic veno-occlusive disease
Opinion receipt date	25 June 2004
Date of Commission Decision	29 July 2004

Active substance	Ciclosporin (inhalation use)
Sponsor	PARI Aerosol Research Institute
Orphan Indication	Prevention of graft rejection after lung transplantation
Opinion receipt date	25 June 2004
Date of Commission Decision	29 July 2004

Active substance	Ciclosporin (inhalation use)
Sponsor	PARI Aerosol Research Institute
Orphan Indication	Treatment of graft rejection after lung transplantation
Opinion receipt date	25 June 2004
Date of Commission Decision	29 July 2004

Active substance	HLA-B27-derived peptide (amino acid 125-138)
Sponsor	LYNKEUS BioTech GmbH
Orphan Indication	Treatment of autoimmune uveitis
Opinion receipt date	9 August 2004
Date of Commission Decision	2 September 2004

Active substance	Anti epidermal growth factor receptor antibody h-R3
Sponsor	Oncoscience AG
Orphan Indication	Treatment of glioma
Opinion receipt date	9 August 2004
Date of Commission Decision	2 September 2004

Active substance	5,10-methylene-tetrahydrofolic acid
Sponsor	Interface International Consultancy Ltd
Orphan Indication	Treatment of pancreatic cancer in combination with 5-fluorouracil
Opinion receipt date	9 August 2004
Date of Commission Decision	2 September 2004

Active substance	Pancreatic enzymes (cross linked enzyme crystal lipase, protease, amylase)
Sponsor	Dr Falk Pharma GmbH
Orphan Indication	Treatment of malabsorption due to exocrine pancreatic enzyme insufficiency
Opinion receipt date	9 August 2004
Date of Commission Decision	2 September 2004

Active substance	Homoharringtonine
Sponsor	Stragen France SAS
Orphan Indication	Treatment of chronic myeloid leukaemia
Opinion receipt date	9 August 2004
Date of Commission Decision	2 September 2004

Active substance	Heparin sodium
Sponsor	Prof. Dr. Seeger
Orphan Indication	Treatment of idiopathic pulmonary fibrosis
Opinion receipt date	9 August 2004
Date of Commission Decision	2 September 2004

Active substance	Sodium dichloroacetate
Sponsor	EBD Group
Orphan Indication	Treatment of systemic monochloroacetate poisoning
Opinion receipt date	9 August 2004
Date of Commission Decision	2 September 2004

Active substance	Recombinant human interleukin-21
Sponsor	Novo Nordisk A/S
Orphan Indication	Treatment of renal cell carcinoma
Opinion receipt date	9 August 2004
Date of Commission Decision	2 September 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

Overview of Designated Orphan Medicinal Products that have been the subject of a Centralised Application for Marketing Authorisation submitted since July 2003

Active substance	***Sponsor/applicant***	***EU Designation Number & Date of Orphan Designation***	***Designated Orphan Indication***
Autologous renal cell tumor vaccine (Reniale)	Liponova GmbH	EU/3/02/116 21/10/2002	Treatment of renal cell carcinoma
Rubitecan (Orathecin)	EuroGen Pharmaceuticals Limited	EU/3/03/145 10/06/2003	Treatment of pancreatic cancer
Sodium oxybate (Xyrem)	Celltech Pharmaceuticals Limited	EU/3/02/131 03/02/2003	Treatment of narcolepsy