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

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PRESS RELEASE
Committee for Orphan Medicinal Products
November 2004 Meeting

The fifty-first meeting of the Committee for Orphan Medicinal Products (COMP) took place on 10-11 November 2004.

COMP Opinions

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

- 17-allylamino-17-demethoxygeldanamycin, from Wainwright Associates Limited, for treatment of **multiple myeloma** (review time: 63 days).
- Sabarubicin, from Menarini Ricerche S.p.A, for treatment of **small cell lung cancer** (review time: 88 days).
- Recombinant histidine-tagged idiotype immunoglobulin Fab fragment of clonal B-cell receptors, from CellGenix Technologie Transfer GmbH, for treatment of **follicular lymphoma** (review time: 63 days).
- Recombinant histidine-tagged idiotype immunoglobulin Fab fragment of clonal B-cell receptors, from CellGenix Technologie Transfer GmbH, for treatment of **mantle cell lymphoma** (review time: 63 days).
- Recombinant histidine-tagged idiotype immunoglobulin Fab fragment of clonal B-cell receptors, from CellGenix Technologie Transfer GmbH, for treatment of **multiple myeloma** (review time: 63 days).
- Val-Leu-Gln-Glu-Leu-Asn-Val-Thr-Val (Pr1 nanopeptide, sequence 169-177, of proteinase 3), from Accelsiors CRO & Consultancy Services GmbH, for treatment of **acute myeloid leukaemia** (review time: 88 days).
- Val-Leu-Gln-Glu-Leu-Asn-Val-Thr-Val (Pr1 nanopeptide, sequence 169-177, of proteinase 3), from Accelsiors CRO & Consultancy Services GmbH, for treatment of **chronic myeloid leukaemia** (review time: 88 days).
- Val-Leu-Gln-Glu-Leu-Asn-Val-Thr-Val (Pr1 nanopeptide, sequence 169-177, of proteinase 3), from Accelsiors CRO & Consultancy Services GmbH, for treatment of **myelodysplastic syndromes** (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 The COMP noted that two applications for orphan medicinal product designation were withdrawn by sponsors.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 fourteen positive decisions on orphan designation[1] since the last COMP meeting on 6-7 October 2004 (see Annex 1).

[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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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*
85	69	17	2	-	58

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.

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 7-8 December 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.

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

Active substance	l, 1´-[1,4-phenylenebis (methylene)]-bis-1,4,8,11-tetraazacyclotetradecane
Sponsor	Orphix Consulting GmbH
Orphan Indication	Treatment to mobilize progenitor cells prior to stem cell transplantation
Opinion receipt date	23/9/2004
Date of Commission Decision	20/10/2004

Active substance	Adeno-associated viral vector containing the human gamma-sarcoglycan gene
Sponsor	Généthon
Orphan Indication	Treatment of gamma-sarcoglycanopathy
Opinion receipt date	23/9/2004
Date of Commission Decision	21/10/2004

Active substance	Biotinylated anti-tenascin monoclonal antibody for use with 90-Yttrium
Sponsor	Sigma Tau Industrie Farmaceutiche Riunite S.p.A
Orphan Indication	Treatment of glioma
Opinion receipt date	23/9/2004
Date of Commission Decision	20/10/2004

Active substance	Deferoxamine mesilate
Sponsor	Neuraxo Biotec GmbH
Orphan Indication	Treatment of traumatic spinal cord injury
Opinion receipt date	23/9/2004
Date of Commission Decision	20/10/2004

Active substance	Deuterium oxide
Sponsor	BDD Berolina Drug Development GmbH
Orphan Indication	Treatment of pancreatic cancer
Opinion receipt date	23/9/2004
Date of Commission Decision	20/10/2004

Active substance	Dexamethasone sodium phosphate encapsulated in human erythrocytes
Sponsor	Dideco S.p.A.
Orphan Indication	Treatment of cystic fibrosis
Opinion receipt date	23/9/2004
Date of Commission Decision	20/10/2004

Active substance	Doxorubicin polyisohexylcyanoacrylate nanoparticles
Sponsor	BioAlliance Pharma
Orphan Indication	Treatment of hepatocellular carcinoma
Opinion receipt date	23/9/2004
Date of Commission Decision	21/10/2004

Active substance	Homoharringtonine
Sponsor	Stragen France SAS
Orphan Indication	Treatment of acute myeloid leukaemia
Opinion receipt date	23/9/2004
Date of Commission Decision	20/10/2004

Active substance	Recombinant human insulin-like growth factor-I/recombinant human insulin-like growth factor binding protein-3
Sponsor	Insmed Incorporated
Orphan Indication	Treatment of Leprechaunism
Opinion receipt date	23/9/2004
Date of Commission Decision	21/10/2004

Active substance	Recombinant human insulin-like growth factor-I/recombinant human insulin-like growth factor binding protein-3
Sponsor	Insmed Incorporated
Orphan Indication	Treatment of Rabson-Mendenhall syndrome
Opinion receipt date	23/9/2004
Date of Commission Decision	21/10/2004

Active substance	Recombinant human insulin-like growth factor-I/recombinant human insulin-like growth factor binding protein-3
Sponsor	Insmed Incorporated
Orphan Indication	Treatment of Type A extreme insulin resistance syndrome
Opinion receipt date	23/9/2004
Date of Commission Decision	21/10/2004

Active substance	Recombinant human insulin-like growth factor-I/recombinant human insulin-like growth factor binding protein-3
Sponsor	Insmed Incorporated
Orphan Indication	Treatment of Type B extreme insulin resistance syndrome
Opinion receipt date	23/9/2004
Date of Commission Decision	20/10/2004

Active substance	Rufinamide
Sponsor	Eisai Limited
Orphan Indication	Treatment of Lennox-Gastaut syndrome
Opinion receipt date	23/9/2004
Date of Commission Decision	20/10/2004

Active substance	Sitaxentan sodium
Sponsor	PPD Global Ltd.
Orphan Indication	Treatment of pulmonary arterial hypertension and chronic thromboembolic pulmonary hypertension
Opinion receipt date	23/9/2004
Date of Commission Decision	21/10/2004

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

- update since the last COMP meeting on 6-7 October 2004 -

Active substance	***Sponsor/applicant***	***EU Designation Number & Date of Orphan Designation***	***Designated Orphan Indication***
Sinapultide, dipalmitoylphosphatidylcholine, palmitoyloleoylphosphatidylglycerol and palmitic acid (Surfaxin)	GMG BioBusiness Ltd.	EU/3/04/216 29/07/2004	Prevention of respiratory distress syndrome in premature neonates of less than 32 weeks of gestational age.
		EU/3/04/217 29/07/2004	Treatment of respiratory distress syndrome in premature neonates of less than 37 weeks of gestational age.
Decitabine (Dacogen)	Eurogen Pharmaceuticals Limited	EU/3/03/135 14/02/2003	Treatment of myelodysplastic syndromes.