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

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PRESS RELEASE
Committee for Orphan Medicinal Products
September 2006 Meeting

The seventy-first meeting of the Committee for Orphan Medicinal Products (COMP) took place on 5-6 September 2006.

COMP Opinions

The Committee adopted 9 positive opinions on orphan medicinal product designation during this meeting:

- 5-(2,6-Difluoro-phenoxy)-3(R,S)-{2(S)-[2(S)-(3-methoxycarbonyl-2(S)-{3-methyl-2(S)-[(quinoline-2-carbonyl)-amino]-butyrylamino}-propionylamino)-3-methyl-butyrylamino]-propionylamino}-4-oxo-pentanoic acid methyl ester, from Theraptosis, **for treatment of neonatal brain injury** (review time: day 59)
- Adenoviral vector containing human p53 gene, from Gendux Aktiebolag, **for treatment of Li Fraumeni Syndrome,** (review time: day 87)
- Arimoclomol, from Wainwright Associates Ltd, **for treatment of amyotrophic lateral sclerosis,** (review time: day 59)
- Doxorubicin hydrochloride (liposomal), from GP-Pharm S.A., **for treatment of soft tissue sarcoma** (review time: day 59)
- Genetically modified allogeneic (human) tumour cells for the expression of IL-7, GM-CSF, CD80 and CD154, in fixed combination with a DNA-based double stem loop immunomodulator (dSLIM), from MOLOGEN AG, **for treatment of renal cell carcinoma** (review time: day 87)
- Heparin-binding epidermal growth factor-like growth factor (HB-EGF), amino acids 74-148, from Dr Michael Moore, **for prevention of necrotizing enterocolitis** (review time: day 59)
- Human cytomegalovirus immunoglobulin, from Biotest Pharma GmbH, **for prevention of congenital cytomegalovirus infection following primary cytomegalovirus infection,** (review time: day 59)
- L-asparaginase encapsulated in erythrocytes, from Erytech Pharma S.A., **for treatment of acute lymphoblastic leukaemia** (review time: day 59)
- Recombinant fusion protein consisting of the extracellular portion of CD95 fused to the Fc part of a human IgG1 molecule, from Apogenix GmbH, **for prevention of Graft-versus-Host disease** (review time: day 59)

Prior to this meeting, the Committee adopted one positive opinion via written procedure on 24 July 2006:

- Mecasermin rinfabate, from ROP Pharma AB, **for prevention of retinopathy of prematurity in neonates of less than 32 weeks of gestational age** (review time: day 43)

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Withdrawals of Orphan Medicinal Product Applications

The COMP noted that two applications for orphan medicinal product designation were withdrawn by the sponsors during evaluation phase of the procedure.

Overview of orphan designation procedures

The European Commission granted sixteen positive decisions on orphan designation[1] since the last COMP meeting on 11-12 July 2006 (see Annex 1).

The status of orphan designation procedures, to date for 2006, is summarised below:

Applications submitted	*Positive COMP Opinions*	*Applications withdrawn*	*Appeals ongoing*	*Final negative COMP Opinions*	*Designations granted by Commission*
73	63	13	-	2	55

An overview of orphan designation procedures for 2000-2005 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 3-4 October 2006.

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.europa.eu.

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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.

Orphan Medicinal Product Designations received since the September 2006 COMP Meeting

Active substance	2-(4-(diethylamino) phenyl)-6-methyl-2H-benzo[d][1,2,3] triazol-5-amine
Sponsor	Vastox Plc
Orphan Indication	Treatment of Duchenne muscular dystrophy
Opinion receipt date	28 June 2006
Date of Commission Decision	25 July 2006

Active substance	4-[123I] iodo-L-phenylalanine
Sponsor	Dr. Andreas Kluge
Orphan Indication	diagnosis of glioma
Opinion receipt date	28 June 2006
Date of Commission Decision	25 July 2006

Active substance	Amikacin sulfate (liposomal)
Sponsor	Morgan Lewis & Bockius
Orphan Indication	Treatment Pseudomonas aeruginosa lung infection in cystic fibrosis
Opinion receipt date	28 June 2006
Date of Commission Decision	25 July 2006

Active substance	Autologous CD34+ cells transfected with retroviral vector containing the human gp91 (phox) gene
Sponsor	Vision 7 GmbH
Orphan Indication	Treatment of chronic granulomatous disease
Opinion receipt date	28 July 2006
Date of Commission Decision	28 August 2006

Active substance	Aviptadil
Sponsor	mondoBIOTECH Laboratories Anstalt
Orphan Indication	Treatment of acute lung injury
Opinion receipt date	28 July 2006
Date of Commission Decision	28 August 2006

Active substance	Becatecarin
Sponsor	Helsinn Birex Pharmaceuticals Ltd
Orphan Indication	Treatment of cancers of the biliary tree
Opinion receipt date	28 June 2006
Date of Commission Decision	25 July 2006

Active substance	Cardiotrophin-1
Sponsor	Digna Biotech S.L
Orphan Indication	Prevention of the ischemia/perfusion injury associated with solid organ transplantation procedure
Opinion receipt date	28 July 2006
Date of Commission Decision	28 August 2006

Active substance	Cholest-4-en-3-one, oxime
Sponsor	Trophos SA
Orphan Indication	Treatment of amyotrophic lateral sclerosis
Opinion receipt date	28 July 2006
Date of Commission Decision	28 August 2006

Active substance	Decitabine
Sponsor	MGI PHARMA LIMITED
Orphan Indication	Treatment of acute myeloid leukaemia
Opinion receipt date	24 April 2006
Date of Commission Decision	8 June 2006

Active substance	Human monoclonal antibody against inhibitory killer cell lg-like receptors (1-7 F9)
Sponsor	Novo Nordisk A/S
Orphan Indication	Treatment of acute myeloid leukaemia
Opinion receipt date	28 July 2006
Date of Commission Decision	28 August 2006

Active substance	Human telomerase reverse transcriptase peptide (611-626)
Sponsor	Pharmexa A/S
Orphan Indication	Treatment of pancreatic cancer
Opinion receipt date	28 June 2006
Date of Commission Decision	25 July 2006

Active substance	H-Val-Ile-Val-Lys-Leu-Ile-Pro-Ser-Thr-Ser-Ser-Ala-Val-Asp-Thr-Pro-Tyr-Leu-Asp-Ile-Thr-Tyr-His-Phe-Val-Ala-Gln-Arg-Leu-Pro-Leu-OH
Sponsor	Debioclinic SA
Orphan Indication	Treatment of myasthenia gravis
Opinion receipt date	28 July 2006
Date of Commission Decision	28 August 2006

Active substance	Lestaurtinib
Sponsor	Cephalon UK Ltd
Orphan Indication	Treatment of acute myeloid leukaemia
Opinion receipt date	28 June 2006
Date of Commission Decision	25 July 2006

Active substance	Metastable technetium 99 [99mTc] Demogastrin 2
Sponsor	Biomedica Life Sciences SA
Orphan Indication	Diagnosis of medullary thyroid carcinoma
Opinion receipt date	28 July 2006
Date of Commission Decision	28 August 2006

Active substance	Mecasermin rinfabate
Sponsor	ROP Pharma AB
Orphan Indication	Prevention of retinopathy of prematurity in neonates of less than 32 weeks
Opinion receipt date	28 July 2006
Date of Commission Decision	28 August 2006

Active substance	N-methyl D-(2,3,4,5,6-pentahydroxy-hexyl)-ammonium; 2-(3,5-dichloro-phenyl)-benzoxazole-6-carboxylate
Sponsor	ICON Clinical Research Limited
Orphan Indication	Treatment of familial amyloid polyneuropathy
Opinion receipt date	28 July 2006
Date of Commission Decision	28 August 2006

Overview of Procedures for Orphan Medicinal Product Designation for 2000-2005

Year	*Applications submitted*	*Positive COMP Opinions*	*Applications withdrawn*	*Final negative COMP Opinions*	*Designations granted by Commission*
2005	118	88	30	0	88
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 11-12 July 2006 -

Active substance	***Sponsor/applicant***	***EU Designation Number & Date of Orphan Designation***	***Designated Orphan Indication***
Recombinant human C1-inhibitor (Rhucin)	Pharming Group N.V.	EU/3/01/036 11/05/2001	Treatment of angioedema by C1 inhibitor deficiency
Ecteinascidin 743(Yondelis)	PharmaMar SA Sociedad Unipersonal	EU/3/01/039 30/05/2001	Treatment of soft tissue sarcoma