



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

12 September 2007
EMEA/COMP/420207/2007

PRESS RELEASE
Committee for Orphan Medicinal Products
September 2007 Meeting

The eighty-second meeting of the Committee for Orphan Medicinal Products (COMP) took place on 11-12 September 2007.

The Committee adopted its 500th opinion on orphan designation at this meeting. This is a clear sign of the successful implementation of the orphan regulation. It is also a strong indication of the continuous effort of all stakeholders and the commitment and dedication of the COMP and the EMEA.

COMP Opinions for Orphan Medicinal Product Designation

The Committee adopted 17 positive opinions on orphan medicinal product designation during this meeting:

- 4-[3,5-bis(trimethylsilyl)benzamido] benzoic acid from Quintiles Ireland Ltd, **for treatment of hepatocellular cancer** (review time: day 62)
- 4-ethoxy-2-(piperazin-1-yl)-7-(pyridin-4-yl)-5H-pyrimido[5,4-b]indol from Curacyte Discovery GmbH, **for treatment of chronic lymphocytic leukaemia** (review time: day 62)
- Adenovirus associated viral vector serotype 4 containing the human RPE65 gene from Centre Hospitalier Universitaire de Nantes, **for treatment of retinitis pigmentosa** (review time: day 62)
- Adenovirus associated viral vector serotype 4 containing the human RPE65 gene from Centre Hospitalier Universitaire de Nantes, **for treatment of Leber's congenital amaurosis** (review time: day 62)
- Alvocidib from Sanofi Aventis, **for treatment of chronic lymphocytic leukaemia** (review time: day 62)
- Amonafide L-malate from INC Research UK Ltd, **for treatment of acute myeloid leukaemia** (review time: day 62)
- Ciclosporin from Novagali Pharma SA, **for treatment of Herpes simplex virus stromal keratitis** (review time: day 62)
- Ciclosporin from Novagali Pharma SA, **for prevention of corneal graft rejection** (review time: day 61)
- Everolimus from Novartis Europharm Limited, **for treatment of gastro-entero-pancreatic neuroendocrine tumours** (review time: day 90)
- Human autologous bone-forming cells derived from bone marrow stem cells from Bone Therapeutics SA, **for treatment of non-traumatic osteonecrosis** (review time: day 90)

- Interferon gamma from Foundation for Fatal Rare Diseases, **for treatment of idiopathic pulmonary fibrosis** (review time: day 62)
- Iodine (¹³¹I) Chlorotoxin from he Weinberg Group LLC, **for treatment of glioma** (review time: day 62)
- Isofagomine tartrate from Amicus Therapeutics UK Ltd, **for treatment of Gaucher disease** (review time: day 62)
- Lenalidomide from Celgene Europe Limited, **for treatment of chronic lymphocytic leukaemia** (review time: day 62)
- Mercaptopurine (oral liquid) from Only For Children Pharmaceuticals, **for treatment of acute lymphoblastic leukaemia** (review time: day 62)
- Methotrexate (oral liquid) from Only for Children Pharmaceuticals, **for treatment of acute lymphoblastic leukaemia** (review time: day 62)
- Polihexanide from S.I.F.I. Società Industria Farmaceutica Italiana S.p.A., **for treatment of Acanthamoeba keratitis** (review time: day 62)

Two oral explanations on orphan medicinal product designation took place during the meeting.

Prior to this meeting, the Committee adopted one positive opinion via written procedure on 30 July 2007:

- Aviptadil from mondoBIOTECH Laboratories, **for treatment of sarcoidosis** (review time: day 95)

Withdrawals of Orphan Medicinal Product Applications

The COMP noted that two applications for orphan medicinal product designation were withdrawn during evaluation.

Overview of orphan designation procedures

The European Commission granted 10 positive decisions on orphan designation¹ since the last COMP meeting on 24-25 July 2007 (see Annex 1).

The status of orphan designation procedures, to date for 2007, is summarised below:

<i>Applications submitted</i>	<i>Positive COMP Opinions</i>	<i>Applications withdrawn</i>	<i>Appeals ongoing</i>	<i>Final negative COMP Opinions</i>	<i>Designations granted by Commission</i>
90	69	15	1	-	45

An overview of orphan designation procedures for 2000-2006 is provided in Annex 2.

Further information on designated orphan medicinal products is publicly available in the form of summarised COMP Opinions², which the Agency routinely publishes following adoption of the respective decisions on orphan designation by the European Commission.

¹ Details of all orphan designations granted to date by the European Commission are entered in the Community Register of Orphan Medicinal Products (http://ec.europa.eu/enterprise/pharmaceuticals/index_en.htm)

² These documents are available on the EMEA web-site

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 9-10 October 2007.

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>.

For further information, please contact:

Martin Harvey Allchurch, EMEA press officer

Tel. (+44-20) 74 18 84 27, E-mail: press@emea.europa.eu.

**Orphan Medicinal Product Designations received
since the July 2007 COMP Meeting**

Active substance	Arsenic trioxide
Sponsor	Cephalon Europe
Orphan Indication	Treatment of acute myeloid leukaemia
Opinion receipt date	9 July 2007
Date of Commission Decision	2 August 2007

Active substance	Ciprofloxacin (inhalation use)
Sponsor	Bayer HealthCare AG
Orphan Indication	Treatment of cystic fibrosis
Opinion receipt date	9 July 2007
Date of Commission Decision	3 August 2007

Active substance	Dihydroartemisinin, piperaquine
Sponsor	Sigma Tau Industrie Farmaceutiche Riunite S.p.A
Orphan Indication	Treatment of malaria
Opinion receipt date	9 July 2007
Date of Commission Decision	3 August 2007

Active substance	Eltrombopag olamine
Sponsor	GlaxoSmithKline Research & Development Limited
Orphan Indication	Treatment of idiopathic thrombocytopenic purpura
Opinion receipt date	9 July 2007
Date of Commission Decision	3 August 2007

Active substance	Human plasminogen
Sponsor	Kedrion S.p.A.
Orphan Indication	Treatment of ligneous conjunctivitis
Opinion receipt date	9 July 2007
Date of Commission Decision	3 August 2007

Active substance	L-threo-3,4-dihydroxyphenylserine
Sponsor	The Weinberg Group LLC
Orphan Indication	Treatment of orthostatic hypotension in patients with multiple system atrophy
Opinion receipt date	9 July 2007
Date of Commission Decision	2 August 2007

Active substance	L-threo-3,4-dihydroxyphenylserine
Sponsor	The Weinberg Group LLC
Orphan Indication	Treatment of orthostatic hypotension in patients with pure autonomic failure
Opinion receipt date	9 July 2007
Date of Commission Decision	2 August 2007

Active substance	Panobinostat lactate
Sponsor	Novartis Europharm Limited
Orphan Indication	Treatment of cutaneous T-Cell Lymphoma
Opinion receipt date	9 July 2007
Date of Commission Decision	2 August 2007

Active substance	Pyridoxalated hemoglobin polyoxyethylene
Sponsor	Curacyte AG
Orphan Indication	Treatment of cardiogenic shock
Opinion receipt date	9 July 2007
Date of Commission Decision	2 August 2007

Active substance	Recombinant human soluble Fc-gamma receptor I I b
Sponsor	SuppreMol GmbH
Orphan Indication	Treatment of idiopathic thrombocytopenic purpura
Opinion receipt date	9 July 2007
Date of Commission Decision	2 August 2007

**Overview of Procedures for Orphan Medicinal Product Designation
for 2000-2006**

<i>Year</i>	<i>Applications submitted</i>	<i>Positive COMP Opinions</i>	<i>Applications withdrawn</i>	<i>Final negative COMP Opinions</i>	<i>Designations granted by Commission</i>
2006	104	81	20	2	80
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 24-25 July 2007-

<i>Active substance</i>	<i>Sponsor/applicant</i>	<i>EU Designation Number & Date of Orphan Designation</i>	<i>Designated Orphan Indication</i>
Icatibant acetate (Firazyr)	Jerini AG	EU/3/03/133 17/02/2003	Treatment of angioedema
Idebenone (Sovrima)	Santhera Pharmaceutiicals (Deutschland AG	EU/3/04/183 08/03/2004	Treatment of Fredreich's ataxia
Ciclosporin (Vekacia)	Novagali Pharma SA	EU/3/06/360 06/04/2006	Treatment of vernal keratoconjunctivitis