



10 January 2007  
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**PRESS RELEASE**  
**Committee for Orphan Medicinal Products**  
**January 2007 Meeting**

The seventy-fifth meeting of the Committee for Orphan Medicinal Products (COMP) took place on 9-10 January 2007. The Committee was delighted to officially welcome two COMP members from the new Member States. An updated list of COMP membership is provided in Annex 1.

**COMP Opinions**

The Committee adopted 6 positive opinions on orphan medicinal product designation during this meeting:

- Artesunate, from ACE Pharmaceuticals BV, **for treatment of malaria** (review time: day 62)
- Autologous dendritic cells pulsed with autologous cell lysate, from Dorian Regulatory Affairs BV, **for treatment of glioma** (review time: day 62)
- Ex-vivo cultured adult human mesenchymal stem cells, from Quintiles UK Limited, **for treatment of Graft-versus-Host disease** (review time: day 62)
- HLA class I/II binding tumour associated peptides (ADF-APO-CCN-GUC-K67-MET-MMP-MUC-RGS), from Immatics Biotechnologies GmbH, **for treatment of renal cell carcinoma** (review time: day 62)
- Idebenone, from Santhera Pharmaceuticals (Deutschland) AG, **for treatment of Leber's hereditary optic neuropathy** (review time: day 90)
- Recombinant human C1-inhibitor, from Pharming Group N.V., **for prevention of delayed graft function after solid organ transplantation** (review time: day 90)

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

**Withdrawals of Orphan Medicinal Product Applications**

The COMP noted that four applications for orphan medicinal product designation were withdrawn by the sponsor during evaluation phase of the procedure.

**Overview of orphan designation procedures**

The European Commission granted four positive decisions on orphan designation<sup>1</sup> since the last COMP meeting on 5-6 December 2006 (see Annex 2).

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<sup>1</sup> 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](http://ec.europa.eu/enterprise/pharmaceuticals/index_en.htm))

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Applications withdrawn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>
-	6	3	-	-	-

An overview of orphan designation procedures for 2000-2006 is provided in Annex 3.

Further information on designated orphan medicinal products is publicly available in the form of summarised COMP Opinions<sup>2</sup>, 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 4.

### **Date of next COMP meeting**

The next COMP meeting will be held on 6-7 February 2007.

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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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<sup>2</sup> These documents are available on the EMEA web-site.

### Composition of the Committee for Orphan Medicinal Products

Chair: Kerstin Westermark  
EMA contact: Agnès SAINT RAYMOND

#### Members

- Brigitte BLÖCHL-DAUM (Austria)
- Andrew BORG (Malta)
- Heidrun BOSCH TRABERG (Denmark)
- Birthe BYSKOV HOLM (patients' organisation representative) (*vice-chairman*)
- Yann LE CAM (patients' organisation representative)
- Ana CORRÊA NUNES (Portugal)
- Bożenna DEMBOWSKA-BAGIŃSKA (Poland)
- Julia DUNNE (EMA representative)
- Judit EGGENHOFER (Hungary)
- Rembert ELBERS (Germany)
- Pauline EVERS (patients' organisation representative)
- Lars GRAMSTAD (Norway)
- Emmanuel HÉRON (France)
- Detelina IVANOVA (Bulgaria)
- Ioannis KKOLOS (Cyprus)
- Kateřina KUBÁČKOVÁ (Czech Republic)
- Magdaléna KUŽELOVÁ (Slovakia)
- André LHOIR (Belgium)
- David LYONS (EMA representative)
- Greg MARKEY (United Kingdom)
- Aušra MATULEVIČIENĖ (Lithuania)
- Henri METZ (Luxembourg)
- Martin MOŽINA (Slovenia)
- Kristina PAVLOVSKA (Latvia)
- Veijo SAANO (Finland)
- Patrick SALMON (Ireland)
- Miranda SIOUTI (Greece)
- Daniela STANCIU (Romania)

**Orphan Medicinal Product Designations received  
since the December 2006 COMP Meeting**

Active substance	4,7,10,13,16,19-docosahexaenoic acid
Sponsor	Jose Manuel Cela Lopez
Orphan Indication	Treatment of retinitis pigmentosa
Opinion receipt date	17 October 2006
Date of Commission Decision	3 November 2006

Active substance	Forodesine hydrochloride
Sponsor	Napp Pharmaceuticals Research Limited
Orphan Indication	Treatment of acute lymphoblastic leukaemia
Opinion receipt date	20 November 2006
Date of Commission Decision	18 December 2006

Active substance	Paclitaxel (micellar)
Sponsor	Oasmia Pharmaceutical AB
Orphan Indication	Treatment of ovarian cancer
Opinion receipt date	20 November 2006
Date of Commission Decision	18 December 2006

Active substance	Tazarotene
Sponsor	Orfagen
Orphan Indication	Treatment of congenital ichthyoses
Opinion receipt date	20 November 2006
Date of Commission Decision	18 December 2006

**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 6-7December 2006-*

<i>Active substance</i>	<i>Sponsor/applicant</i>	<i>EU Designation Number &amp; Date of Orphan Designation</i>	<i>Designated Orphan Indication</i>
gusperimus trihydrochloride (Spanidin)	Euro Nippon Kayaku GmbH	EU/3/01/034 29/03/2001	Treatment of Wegener's granulomatosis