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

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PRESS RELEASE
Committee for Orphan Medicinal Products
April 2006 Meeting

The sixty-seventh meeting of the Committee for Orphan Medicinal Products (COMP) took place on 4-5 April 2006.

After three years, this was the last meeting of the current COMP mandate. The Committee thanked warmly Prof Josep Torrent-Farnell and Mr Yann Le Cam for their dedication as Chair and Vice-Chair. Also the following members who will leave the COMP have been thanked for their successful contribution to the work of the Committee: Prof. Fernando de Andrés-Trelles, Mr Alastair Kent, Ms. Kristina Pavlovska, Dr Harrie Seeverens, Dr George Stathopoulos, Dr Algirdas Utkus and Dr Jolanta Więckowska. The new three-year mandate starts with the meeting in May.

COMP Opinions

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

- 1-deoxygalactonojirimycin hydrochloride, from Amicus Therapeutics UK Ltd, for treatment of **Fabry disease** (review time: day 87)
- Bilayer engineered skin composed of keratinocytes from the patient (autologous) and fibroblasts from a donor (allogeneic) embedded in a plasma matrix, from Cellerix S.L., for treatment of **epidermolysis bullosa** (review time: day 45)
- Decitabine, from MGI Pharma Limited, for treatment of **acute myeloid leukaemia** (review time: day 45)
- Heparin sodium, from Ockham Biotech Limited, for treatment of **cystic fibrosis** (review time: day 45)
- Hydrocortisone (modified release tablet), from DuoCort AB, for treatment of **adrenal insufficiency** (review time: day 87)
- Mecasermin, from Tercica Europe Limited, for treatment of **primary insulin-like growth factor-1 deficiency due to molecular or genetic defects** (review time: day 87)
- Methoxsalen, from Johnson & Johnson Medical Ltd, for treatment of **Graft-versus-Host disease** (review time: day 45)
- Nilotinib, from Novartis Europharm limited, for treatment of **chronic myeloid leukaemia** (review time: day 45)
- Recombinant P-selectin glycoprotein immunoglobulin, from RJM Consultancy Ltd, for prevention of **post transplantation graft dysfunction** (review time: day 45)

Six oral explanations took place during the meeting. The COMP noted that three applications for orphan medicinal product designation were withdrawn by the sponsor during the evaluation phase of the procedure.

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Two negative opinions on orphan medicinal product designation were adopted. These opinions will be forwarded to the sponsors, who may submit detailed grounds for appeal within 90 days of receipt of the opinion.

Overview of orphan designation procedures

The European Commission granted no positive decisions on orphan designation[1] since the last COMP meeting on 7-8 March 2006.

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*
27	28	6	-	-	15

An overview of orphan designation procedures for 2000-2005 is provided in Annex 1.

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 2.

Date of next COMP meeting

The next COMP meeting will be held on 15-16 May 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.eu.int/

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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.

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 7-8 March 2006 -

Active substance	***Sponsor/applicant***	***EU Designation Number & Date of Orphan Designation***	***Designated Orphan Indication***
3-(4'aminoisoindoline-1'-one)-1-piperidine-2,6-dione (Lenalidomide – Celgene Europe)	Celgene Europe Limited	EU/3/03/177 12/12/2003	Treatment of multiple myeloma
Imatinib mesylate (Glivec)	Novartis Europharm Limited	EU/3/05/320 28/10/2005	Treatment of chronic eosinophilic leukaemia and the hypereosinophilic syndrome