



19 April 2000
EMA/COMP/5/00

PRESS RELEASE

Inaugural Meeting of the Committee for Orphan Medicinal Products

The Committee for Orphan Medicinal Products (COMP), established under Regulation (EC) 141/2000¹ of 16 December 1999, held its Inaugural Meeting on 17 April 2000.

The Executive Director of the European Agency for Evaluation of Medicinal Products (EMA) welcomed the members of the new Committee and chaired the meeting, pending election of a Chairperson at the next Committee meeting.

The European Commission's representative underlined the fact that this is the first institutional Committee of the European Union where representatives of patient organisations are full members. The COMP comprises twenty-one members: one member nominated by each Member State, three Members nominated by the European Commission to represent patients' organisations and three members nominated by the European Commission on the basis of a recommendation from the EMA. The European Commission finalised the membership of the COMP on 14 April 2000 (Annex 1).

The COMP will be responsible for examining all applications for orphan medicinal product designation, submitted in accordance with Regulation (EC) 141/2000 of 16 December 2000 and the Commission Implementing Regulation, adoption of which is expected on 27 April 2000². This new Committee will also be responsible for advising the European Commission on the establishment and development of a policy on orphan medicinal products in the European Union and will assist the Commission in drawing up detailed guidelines and liaising internationally on matters relating to orphan medicinal products.

During this meeting, the Committee provisionally discussed the Rules of Procedure of the Committee and agreed on the application of the EMA Code of Conduct to guarantee the independence of Committee members and experts. The Committee fully endorsed a policy of transparency, with regular Press Releases and the development of a communication policy directed towards patient organisations.

The Committee endorsed a draft Commission guideline (ENTR/6283/00) for the format and content of applications for designation as orphan medicinal products. This draft guideline is due for release by the European Commission in May, for a 5 month consultation period. Sponsors planning to submit applications for orphan medicinal product designation will then be able to use the application form annexed to it. To date, 31 sponsors have informed the EMA of their intention to apply for orphan medicinal product designation.

The Committee fully supported the principle that the EMA continues to grant fee exemptions to those six centrally authorised medicinal products, which are indicated for patients with rare diseases and have received fee exemptions in the past. The Committee reviewed the budgetary situation and noted that a special contribution of €1,000,000 is expected from the European Budgetary Authority (Annex 2).

An initial debate on the designation procedure for orphan medicinal products was held by the Committee, with a preliminary discussion on how to introduce the appropriate expertise into the system. The Committee aims to further define the designation procedure at their next meeting to permit validation of applications by the EMA.

The next meeting of the COMP will be held on 15 May 2000. Following that meeting, dates of COMP meetings in 2000 and 2001 will be published by the EMA.

¹ Official Journal of the European Communities, L 18/1, 22.1.2000

² The draft text of the Commission Regulation, is available on the internet: <http://pharmacos.eudra.org>

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.eudra.org/emea.html>

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Ελλάδα	Prof. Thrassyvoulos KEPHALAS
España	Prof. Josep TORRENT-FARNELL
France	Dr. François MEYER
Ireland	Dr. Brendan BUCKLEY
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Luxembourg/Luxemburg	Mme. Mariette BACKES-LIES
Nederland	Dr. H.J.J. SEEVERENS
Österreich	Prof.Dr. Hans Georg EICHLER
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Suomi/Finland	Dr. Kalle HOPPU
Sverige	Dr. Kerstin WESTERMARK
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EMA Representative	Prof. Jean-Michel ALEXANDRE
EMA Representative	Dr. Mary TEELING
EMA Representative	Prof. Gianmartino BENZI
Representative of Patient Organisation	Mr. Abascal ALONSO
Representative of Patient Organisation	Mr. Yann LE CAM
Representative of Patient Organisation	Mr. Alastair KENT

I. 'ORPHAN DRUGS' THAT RECEIVED FEE EXEMPTIONS PRIOR TO THE ENTRY INTO FORCE OF THE REGULATION (1996-1999)

Product	Company	Indication <i>Number of patients in the EU</i>	Date of Authorisation	Fee Exemptions
Cystagon	Orphan Europe	Treatment of proven nephropathic cystinosis 400-600	23 June 1997	From 1996 to 1999, using its own financial resources, the EMEA has granted some € 600 000 fee exemptions for these products (application fee, variation fee, annual fee). In 2000, estimated fees from these existing 'orphan drugs' is some € 480 000 (annual fees, variation fees).
BeneFIX	Genetics Institute	Control and prevention of haemorrhagic episodes and prophylaxis in haemophilia B patients < 4,000	27 August 1997	
Beromun	Boehringer Ingelheim	Adjunct to surgery for subsequent removal of the tumour so as to prevent or delay amputation, or in the palliative situation, for irresectable soft tissue sarcoma of the limbs 1,250	13 April 1999	
Cerezyme	Genzyme	Long term enzyme replacement therapy in patients with Type I Gaucher disease 1,825	17 November 1997	
Ammonaps	Orphan Europe	Adjunctive therapy in the chronic management of urea cycle disorders 400	8 December 1999	
Quadramet	CIS bio international	For the relief of bone pain in patients with multiple painful osteoblastic skeletal metastases < 2,000	5 February 1998	

II. FEE ESTIMATES FOR NEW ORPHAN DRUGS IN 2000

To date, 31 companies have indicated their intention to apply for orphan status designation to the Committee for Orphan Medicinal Products. On the basis of indications provided by companies, projections are the following:

Type of applications and activities	Estimated fee revenue in 2000
Protocol assistance – 4 requests	€ 240 000
Marketing authorisation – 7 requests	€ 1 680 000
Pre-authorisation Inspections – 4 requests	€ 30 000
Grand total	€ 1 950 000

III. OVERVIEW OF EMEA BUDGETARY NEEDS 2000-2003

Year	2000	2001	2002	2003
Number of orphan medicines	11	14	20	22
Special contribution for partial fee exemptions	€ 1 000 000	€ 1 300 000	€ 1 820 000	€ 2 000 000
Estimated contribution from EU basic subsidy for EMEA meeting and staff costs	€ 500 000	€ 710 000	€ 722 000	€ 736 000

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