The European Agency for the Evaluation of Medicinal Products
Evaluation of Medicines for Human Use

14 July 2000
EMEA/COMP/38/00

PRESS RELEASE

4th Meeting of the Committee for Orphan Medicinal Products

The Committee for Orphan Medicinal Products (COMP) held its fourth meeting on 11 July 2000.

The Committee adopted the first opinions on the designation of orphan medicinal products, two months after the adoption of the EU Implementing Regulation[1]. Three positive opinions were adopted by consensus on applications for orphan medicinal product designation, two for Fabry disease and one for AIDS wasting. These opinions, adopted at day 27 of the 90-day procedure, will now be forwarded to the European Commission for the decision making process. Designated orphan medicinal products will be entered into a Community Register of Orphan Medicinal Products.

The Committee would like to underline the fact that the designation assigns orphan status on epidemiologic or economic criteria for life-threatening or debilitating conditions where there is no satisfactory method of treatment in the Community which has been authorised. Where such methods exist the sponsor must establish that the medicinal product will be of significant benefit to the patients affected by the condition.

Designation of orphan status is not by any means an endorsement for the use of the product in that condition as it does not in any way indicate that the product will satisfy the criteria for the grant of a marketing authorisation. The quality, safety and efficacy of the medicinal product for the proposed therapeutic indication can only be evaluated, as for any medicinal product, once the application for marketing authorisation is submitted.

The Committee heard presentations from Dr. Annie Wolf, Mission des Médicaments Orphelins, Ministry of Health in France, on the background to Regulation (EC) 141/2000 of 16 December 2000 and from Dr. Ségolène Aymé, Director of Research at INSERM (Institut National de la Santé et de la Recherche Medicale) on the OPRHANET database and a number of other databases in the field of rare diseases.

Co-ordinators and experts were appointed by the Committee for a number of upcoming applications. The status of orphan designation procedures, as of 11 July 2000, is summarised in the table below:

Year	*Intent to file Notified*	*Applications submitted*	*Positive COMP Opinions*	*Negative COMP Opinions*	*Designations granted by Commission*
2000	26	24	3	-	-

The next meeting of the COMP will be held on 12-13 September 2000.

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

Contacts for further information:
Evaluation of Medicines for Human Use Unit

Dr. Patrick Le Courtois
Tel. (44-20) 74 18 86 49

or Ms. Melanie Carr
Tel. (44-20) 74 18 85 75
Fax. (44-20) 75 23 70 40

[1] Commission Regulation (EC) No 847/2000 of 27 April 2000 (Official Journal of the European Communities, L 103/5, 28.4.00)