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

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EMEA/COMP/20/00

PRESS RELEASE
3rd Meeting of the Committee for Orphan Medicinal Products

The Committee for Orphan Medicinal Products (COMP) held its third meeting on 15 June 2000, chaired by Prof. Josep Torrent-Farnell.

Mr. Yann Le Cam (Patient Organisation Representative) was elected vice-chairperson of the Committee for a term of three years, renewable once.

The Committee welcomed Dr. Rashmi Shah from the Medicines Control Agency as the new UK member and Dr. Randi Nordal from the Norwegian Medicines Control Authority as an observer.

In follow-up to the in-depth discussion on the procedure for orphan medicinal product designation that was held by the Committee at its May meeting, the Committee adopted a document outlining the general principles of the procedure (EMEA/14222/00, attached in Annex 1). On the basis of this document, a more detailed Standard Operating Procedure will be drafted, which will be made public once finalised.

The Committee further discussed the building up of an expert network on rare diseases and appointed experts for a number of applications. Dr. Domenica Taruscio, COMP member for Italy, presented the Network for Public Health Institutions on Rare Diseases (NEPHIRD), which is coordinated by the Istituto Superiore di Sanità in Italy. Mr. Alastair Kent and Mr. Yann Le Cam, COMP members representing patient organisations, presented proposals on the role that patient groups could play in identifying experts.

To date, a total of 24 applications for orphan medicinal product designation have been submitted to the EMEA. A further 11 sponsors have formally notified the EMEA of their intention to apply for orphan designation and the EMEA has handled queries relating to an additional 20 potential upcoming applications.

The Committee discussed the 10 applications for orphan medicinal product designation for which co-ordinators were appointed at the May meeting. Four of these applications have successfully completed validation and the designation procedure has now started.

Co-ordinators were appointed by the Committee for the applications that were submitted following the May meeting and for those applications which are planned for submission in June and July 2000.

Sponsors are reminded to notify the EMEA of their intention to submit an application at least two months prior to the planned submission date. This notification should take the form of a letter, to the attention of Dr. Patrick Le Courtois at the EMEA, and should include: the name of the product, the proposed indication, the name and address of the sponsor and the planned submission date for the designation application.

In preparing an application for orphan medicinal product designation, sponsors are requested to follow the draft Commission guideline[1] (ENTR/6283/00) on the format and content of applications. Sponsors should clearly substantiate the claims and support the statements made in the application with references where possible. Sponsors are encouraged to request a pre-submission meeting with the EMEA prior to filing.

Sponsors should note that Regulation (EC) 141/2000[2] of 16 December 1999 on orphan medicinal products, applies to medicinal products for human use only. The Regulation does not apply to veterinary medicinal products.

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[1] ENTR/6283/00 is available on the EMEA and Commission (http://dg3.eudra.org) web-sites
[2] Official Journal of the European Communities, L 18/1, 22.1.2000

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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.eudra.org/emea.html

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ANNEX 1

London, 15 June 2000
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Procedure for Orphan Medicinal Product Designation – General Principles

Introduction

Regulation (EC) No 141/2000 of 16 December 1999 lays down a community procedure for the designation of medicinal products as orphan medicinal products and the criteria for designation of orphan status.

The Regulation establishes the Committee for Orphan Medicinal Products (COMP), within the EMEA, which is responsible for examining all applications for orphan medicinal product designation submitted to it in accordance with the Regulation.

Objectives

In examining an application for orphan medicinal product designation the Committee will focus on determining whether the sponsor has established that the designation criteria are met, i.e:

- the life-threatening or debilitating nature of the condition
- the medical plausibility of the proposed therapeutic indication
- the prevalence of the condition in the Community is not more than five in 10,000; or

that it is unlikely the marketing the medicinal product in the Community, without incentives would generate sufficient return to justify the necessary investment

- that no satisfactory method of diagnosis prevention or treatment exists or that if such a method exists that the medicinal product will be of significant benefit to those affected by that condition

In order to assist in the development of a policy on orphan medicinal products an expert network will be built up by the Committee with expert(s) identified as appropriate to be involved in the evaluation of applications for orphan medicinal product designation. Where necessary information will be gathered from Committee members and patient organisations on the clinical setting for treating a particular condition in each of the Member States.

General Principles

Pre-submission

- Sponsors should notify the EMEA of their intention to submit an application as early as possible and at the latest two months prior to the planned submission date.
- In preparing an application for orphan medicinal product designation, sponsors are requested to follow the draft Commission guideline (ENTR/6283/00) for the format and content of applications for designation as orphan medicinal products, available on the EMEA and Commission web-site.
- Two co-ordinators (1 COMP member, 1 EMEA staff member) will be appointed for each application.
- COMP members will be invited to propose experts to be involved in the evaluation as appropriate. The Committee may appoint one or two experts from the EU expert list to be involved in each application, in addition to the co-ordinators, as appropriate.

Submission of the application

- The sponsor will submit the application to the EMEA (1 original + 2 complete copies). Deadlines for submission will be published.

Validation

- The EMEA Secretariat will complete the validation. Where validation issues arise, the EMEA co-ordinator will liase with the COMP co-ordinator, and experts if necessary, to resolve them.

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- In order to synchronise each evaluation with the meetings of the Committee for Orphan Medicinal Products (COMP), validation dates (Day 1, start of the procedure) will be fixed.
- Once the validation process is successfully completed, a time-table for the evaluation will be adopted. The EMEA will immediately forward a copy of the application to all COMP members and the appointed experts.

Evaluation

- During the evaluation phase the EMEA co-ordinator will work very closely with the COMP co-ordinator and appointed expert(s). Teleconference/video conferences or meetings at the EMEA will be set up as necessary.
- The co-ordinators may gather information from Committee members on the disease state, availability of treatments, research status etc.
- The EMEA co-ordinator, in association with the COMP co-ordinator, will prepare a summary report on the application. The summary report will include factual data, a critical review and a conclusion. Where there is a need to organise an oral explanation with the sponsor, this will be highlighted. In this case the report will identify the main issues to be addressed by the sponsor.
- Following agreement between the EMEA co-ordinator and the COMP co-ordinator, the summary report will be circulated to COMP members for comments. Members of COMP will forward comments to EMEA, with other COMP members on copy, in accordance with the adopted time-table.
- At the meeting(s) following circulation of the summary report, COMP will discuss the application together with comments raised. Where possible the expert(s) involved in the application will be invited to attend the COMP discussion.

Opinion

- Before day 90, the COMP adopts its opinion (in English).
- If a negative outcome of the review of the application appears probable the sponsor will be invited for an oral explanation before the COMP prior to adoption of the opinion. The Co-ordinators will prepare a document highlighting the points of disagreement and requests for clarification.
- The opinion may be obtained during a COMP meeting or by written procedure. The COMP opinion, which may be favourable or unfavourable, is, wherever possible, reached by consensus.
  If such consensus cannot be reached, the opinion shall be adopted by a majority of two-thirds (i.e. 14 members) of the COMP.
- The EMEA, taking into account the discussion within the COMP and the conclusions reached, will revise the summary report, which once adopted by the COMP will become the COMP assessment report.

Follow-up to the COMP Opinion

- The EMEA will forward the opinion to the Commission and the sponsor.

Appeal

- In case of a negative opinion, the sponsor may appeal.
- The grounds for appeal must be forwarded to the EMEA within 90 days of receipt of the opinion.
- The EMEA will refer the grounds for appeal to the COMP who will consider whether its opinion should be revised at the first meeting following receipt of the grounds for appeal.

Decision Making

The Decision will be adopted by the Commission, within 30 days of its receipt of the opinion.

Publication in the Register

Upon a favourable decision by the Commission, the designated medicinal product shall be entered in the Community Register of Orphan Medicinal Products.