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

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COMP/16/01 corr.

PRESS RELEASE

9th Meeting of the Committee for Orphan Medicinal Products

The Committee for Orphan Medicinal Products (COMP) held its ninth meeting on 15 January 2001.

The Committee thanked Dr. Jan Renneberg, who is resigning his COMP membership, for his contribution and collaboration and welcomed Dr. Heidrun Bosch-Traberg as the new Danish member.

Three positive opinions on the designation of orphan medicinal products, were adopted by the Committee, for the following conditions:

- Adenovirus infection in immunocompromised patients
- Graft versus Host Disease
- Patent ductus arteriosus in premature neonates of less than 34 weeks gestational age

These opinions, will now be forwarded to the European Commission for the decision making process.

One negative opinion on orphan medicinal product designation was adopted. This opinion will be forwarded to the sponsor, who may submit detailed grounds for appeal within 90 days of receipt of the opinion.

The COMP noted that 6 decisions on orphan designation have been granted by the European Commission[1] since the last COMP in December 2000, see Annex I.

The status of orphan designation procedures, as of 15 January 2001, is summarised in the table below:

Year	*Intent to file Notified*	*Applications submitted*	*Applications withdrawn*	*Positive COMP Opinions*	*Negative COMP Opinions*	*Designations granted by Commission*
2000	31	72	3	26	-	14
2001	1	-	-	3	1	-
Total	*32*	*72*	*3*	*29*	*1*	*14*

The Committee appointed co-ordinators and experts for a number of upcoming applications.

During this meeting, the Committee agreed upon a revision to the Rules of Procedure of the Committee. Article 3, which concerns the election of the Chairperson and Vice-Chairperson, was brought in to line with the Rules of other EMEA Committees. The revised Rules of Procedure (EMEA/8212/00 Rev 1) are available on the EMEA web-site.

The document titled 'Procedure for orphan medicinal product designation - General Principles' (EMEA/14222/00), which was adopted by COMP on 15 June 2000, was reviewed by the Committee in light of the 6 months of experience now gained with the procedure. A deadline of 3 months for response to validation issues by sponsors and a number of other minor changes were incorporated. A copy is provided in Annex II.

The next meeting of the COMP will be held on 8-9 February 2001.

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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://dg3.eudra.org/register/index.htm)

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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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Medicinal products Designated as Orphan Medicinal Products since the December 2000 Press Release

Active substance	Thalidomide
Sponsor	Laboratoires Laphal
Orphan Indication	Treatment of Erythema nodosum leprosum (ENL) or type II lepra reactions
Opinion receipt date	6/11/00
Date of Commission Decision	29/12/00

Active substance	Anagrelide Hydrochloride
Sponsor	Shire Pharmaceutical Development Ltd
Orphan Indication	Treatment of essential thrombocythaemia
Opinion receipt date	6/11/00
Date of Commission Decision	29/12/00

Active substance	Busulfan (intravenous use)
Sponsor	Pierre Fabre Médicament
Orphan Indication	Conditioning treatment prior to hematopoietic progenitor cell transplantation
Opinion receipt date	6/11/00
Date of Commission Decision	29/12/00

Active substance	Nitisinone
Sponsor	Swedish Orphan AB
Orphan Indication	Treatment of tyrosinaemia type 1
Opinion receipt date	6/11/00
Date of Commission Decision	29/12/00

Active substance	Ethyl Eicosapentaenoate
Sponsor	Laxdale Ltd.
Orphan Indication	Treatment of Huntington's disease
Opinion receipt date	6/11/00
Date of Commission Decision	29/12/00

Active substance	Iloprost
Sponsor	Schering AG
Orphan Indication	Treatment of primary and of the following forms of secondary pulmonary hypertension: connective tissue disease pulmonary hypertension, drug-induced pulmonary hypertension, portopulmonary hypertension, pulmonary hypertension associated with congenital heart disease and chronic thromboembolic pulmonary hypertension
Opinion receipt date	6/11/00
Date of Commission Decision	29/12/00

ANNEX II
15 January 2001
EMEA/14222/00 rev 1

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 orphan 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 on the clinical setting for treating a particular condition in each of the Member States will be gathered from Committee members and patient organisations.

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 revised 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-sites.
- 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 major validation issues arise, the EMEA co-ordinator will liase with the COMP co-ordinator, and experts if necessary, to resolve them.
- 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.
- In the event that the EMEA requires additional data, information or clarification to complete its validation, the sponsor will be contacted and asked to respond within a 3-month time limit. If no response from the sponsor is received within this time frame, the sponsor will be asked to submit a new complete application.
- 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.

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 for written/oral explanation from 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 may 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.

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