The European Agency for the Evaluation of Medicinal Products
Pre-authorisation evaluation of medicines for human use

13 September 2001
EMEA/COMP/385/01

PRESS RELEASE

16th Meeting of the Committee for Orphan Medicinal Products

The sixteenth meeting of the Committee for Orphan Medicinal Products (COMP) was held on 6-7 September 2001. Four oral explanations took place during the meeting.

The Committee was pleased to hear that, further to the positive CPMP opinions adopted in March 2001, the first EU orphan marketing authorisations were granted by the European Commission on 3 August 2001. The designated orphan medicinal products Fabrazyme® and Replagal® were authorised for long-term enzyme replacement therapy in patients with a confirmed diagnosis of Fabry disease (α-galactosidase A deficiency).

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

- Chronic lymphocytic leukaemia
- Erythema nodosum lepra (ENL) or type II lepra reactions
- Familial Adenomatous Polyposis (FAP)
- Friedreich's ataxia
- Ileal pouch anal anastomosis (IPAA) related faecal incontinence
- Invasive fungal infections
- Lupus nephritis
- Malignant gastrointestinal stromal tumours
- Multiple myeloma (*3 x opinions*)
- Off-periods in Parkinson's disease not responding to oral treatment
- Severe myoclonic epilepsy in infancy

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. Three applications for orphan medicinal product designation were withdrawn by sponsors.

The European Commission granted three decisions on orphan designation[1] since the last COMP meeting on 17-18 July 2001, see Annex I. The status of orphan designation procedures, as of 7 September 2001, is summarised in the table below:

Intent to file notified	*Applications submitted*	*Applications withdrawn*	*Positive COMP Opinions*	*Negative COMP Opinions*	*Designations granted by Commission*
52	131	22	73	1[2]	52

Sponsors of designated orphan medicinal products are required to submit annual reports to the EMEA on the state of development of the medicinal product. The Committee finalised a draft guideline (COMP/189/01)[3] on the format and content of such annual reports, which is released for 3 months consultation. In preparing annual reports for orphan medicinal products, sponsors are requested to follow the draft guideline.

The next COMP meeting will be a one-day meeting and will take place on 26 October 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://pharmacos.eudra.org/F2/register/index.htm)

2 Of the four negative opinions adopted to date, one is ongoing. Two applications have been subsequently withdrawn and one has resulted in a final positive opinion following appeal.

3 The draft guideline is published on the EMEA web-site: http://www.emea.eu.int

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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.emea.eu.int/

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Medicinal products Designated as Orphan Medicinal Products on 31 July 2001

Active substance	Seocalcitol
Sponsor	Leo Pharmaceutical Products
Orphan Indication	Treatment of hepatocellular carcinoma
Opinion receipt date	18/6/01
Date of Commission Decision	31/7/01

Active substance	1,3-Propanedisulfonic acid, disodium salt
Sponsor	Quintiles Limited
Orphan Indication	Treatment of Systemic Secondary Amyloidosis
Opinion receipt date	18/6/01
Date of Commission Decision	31/7/01

Active substance	Zinc acetate dihydrate
Sponsor	Orphan Europe SARL
Orphan Indication	Treatment of Wilson's disease
Opinion receipt date	18/6/01
Date of Commission Decision	31/7/01