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

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EMEA/COMP/519/01

PRESS RELEASE

18th Meeting of the Committee for Orphan Medicinal Products

The eighteenth meeting of the Committee for Orphan Medicinal Products (COMP) was held on 20-21 November 2001.

During the meeting, representatives of the Non-Governmental Organisation (NGO) Médecins Sans Frontières presented the Drugs for Neglected Diseases Initiative.

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

- Acute lymphoblastic leukaemia
- Acute lung injury
- Diarrhoea associated with intestinal microsporidial infection
- High-grade glioma
- Myelodysplastic syndromes
- Systemic sclerosis

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

One oral explanation took place during the meeting. The COMP noted that three applications for orphan medicinal product designation were withdrawn by sponsors.

The European Commission granted ten decisions on orphan designation[1] since the last COMP meeting on 26 October 2001, see Annex I. The status of orphan designation procedures, as of 21 November 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Intent to file notified	*Applications submitted*	*Applications withdrawn*	*Positive COMP Opinions*	*Negative COMP Opinions*	*Designations granted by Commission*
44	149	27	84	1[2]	70

The Committee finalised a draft 'Points to Consider Document on the Calculation and Reporting of the Prevalence of a Condition for Orphan Designation' (COMP/436/01), which will be released on the EMEA web-site for a 3 months consultation period. An ad-hoc group of experts on epidemiology was convened by COMP to assist in the preparation of this guidance document. Sponsors are advised to consult this 'points to consider' document prior to preparing the prevalence section of an application for orphan medicinal product designation.

The Committee adopted the formal mandate of the COMP Working Group with Interested Parties (COMP-WGIP), which met for the first time in July 2001 (COMP/264/01 - attached in Annex II). The COMP-WGIP, which is composed of EMEA/COMP members and representatives of patient organisations and of the pharmaceutical industry, will work on proposals for improving transparency on orphan activities, optimising the orphan designation procedure and delineating policy recommendations on orphan medicinal products.

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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.

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In follow-up to the first Workshops with Interested Parties, which were held with patient representatives and the pharmaceutical industry in March and April 2001 respectively, a Workshop with Academia and Health Professionals has been scheduled on 24 January 2002.

The next COMP meeting will be held on 17-18 December 2001.

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 in November 2001

Active substance	Abetimus sodium
Sponsor	Icon Clinical Research UK Ltd
Orphan Indication	Treatment of lupus nephritis
Opinion receipt date	17/9/01
Date of Commission Decision	20/11/01

Active substance	Imatinib mesilate
Sponsor	Novartis Europharm Limited
Orphan Indication	Treatment of malignant gastrointestinal stromal tumours
Opinion receipt date	17/9/01
Date of Commission Decision	20/11/01

Active substance	Idebenone
Sponsor	Laboratoires Takeda
Orphan Indication	Treatment of Friedreich's ataxia
Opinion receipt date	17/9/01
Date of Commission Decision	20/11/01

Active substance	Deoxyribose phosphorothioate (5'-tct-ccc-agc-gtg-cgc-cat-3')
Sponsor	Voisin Consulting S.A.R.L.
Orphan Indication	Treatment of chronic lymphocytic leukaemia
Opinion receipt date	17/9/01
Date of Commission Decision	20/11/01

Active substance	Deoxyribose phosphorothioate (5'-tct-ccc-agc-gtg-cgc-cat-3')
Sponsor	Voisin Consulting S.A.R.L.
Orphan Indication	Treatment of multiple myeloma
Opinion receipt date	17/9/01
Date of Commission Decision	20/11/01

Active substance	Thalidomide
Sponsor	Kendle International Limited
Orphan Indication	Treatment of multiple myeloma
Opinion receipt date	17/9/01
Date of Commission Decision	20/11/01

Active substance	Thalidomide
Sponsor	Pharmion Limited
Orphan Indication	Treatment of multiple myeloma
Opinion receipt date	17/9/01
Date of Commission Decision	20/11/01

Active substance	Thalidomide
Sponsor	Pharmion Limited
Orphan Indication	Treatment of Erythema nodosum lepra (ENL) or Type II lepra reactions
Opinion receipt date	17/9/01
Date of Commission Decision	20/11/01

Active substance	Phenylephrine hydrochloride
Sponsor	S.L.A. Pharma (UK) Limited
Orphan Indication	Treatment of ileal pouch anal anastomosis (IPAA) related faecal incontinence
Opinion receipt date	17/9/01
Date of Commission Decision	20/11/01

Active substance	Celecoxib
Sponsor	Pharmacia-Pfizer EEIG
Orphan Indication	Treatment of Familial Adenomatous Polyposis (FAP)
Opinion receipt date	17/9/01
Date of Commission Decision	20/11/01

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COMP WORKING GROUP WITH INTERESTED PARTIES

Mandate and rules of procedure

In relation with its responsibilities according to article 4-2 para b, c and d of Regulation (EC) No 141/2000 and in the framework of its transparency activity within the EMEA, the COMP (Committee for Orphan Medicinal Products), following workshops with Interested Parties on 21 March and 11 April 2001, has decided* on 22-23 May 2001 to create a *COMP Working Group with Interested Parties*.

Mandate

In the interest of public health and with a view towards improving access to medicinal products for patients affected by orphan diseases, the *COMP Working Group with Interested Parties* will prepare proposals for COMP concerning:

- transparency of EMEA/COMP activities related to the orphan designation procedure
- optimisation of the orphan designation procedure
- policy recommendations on orphan medicinal products
- any other topic of interest at the request of the COMP

The *COMP Working Group with Interested Parties* shall agree on a work plan defining priorities of activities to be endorsed by the COMP.

Composition

The *COMP Working Group with Interested Parties* is composed of members from the COMP, the EMEA and Interested Parties.

Interested Parties identified by COMP/EMEA will nominate up to three members by Interested Party.

The *COMP Working Group with Interested Parties* may propose participation of specific experts or observers as necessary.

The members of *COMP Working Group with Interested Parties* shall commit to active participation of the activities of the group.

Meetings will be co-chaired by one COMP representative and one representative from the EMEA staff.

Confidentiality

Confidentiality will be respected by participants of *COMP Working Group with Interested Parties* when information or documents will be identified as confidential by the EMEA.

Report/Interactions

Reports on activities and proposals from *COMP Working Group with Interested Parties* will be presented to the COMP by COMP/EMEA members.

COMP/EMEA members will report to the *COMP Working Group with Interested Parties* on discussions held in COMP relevant to the activities of the *COMP Working Group with Interested Parties*.

* proposal endorsed by the EMEA Management Board on 6 June 2001

Organisation

As far as possible, the *COMP Working Group with Interested Parties* will nominate co-ordinators on specific topics.

Secretariat or the *COMP Working Group with Interested Parties* meetings

Secretariat of the *COMP Working Group with Interested Parties* will be ensured by the EMEA.