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

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PRESS RELEASE

6th Meeting of the Committee for Orphan Medicinal Products

The Committee for Orphan Medicinal Products (COMP) held its sixth meeting on 26-27 October 2000.

The Committee thanked Prof. Thrassyvoulos Kephalas, who is resigning his COMP membership, for his contribution and collaboration and welcomed Dr. George Stathopoulos as the new Greek member. An updated list of COMP members is provided in Annex I.

An Ad Hoc meeting of COMP members was held on 25 October 2000 to prepare contributions for the upcoming EMEA Workshop on Transparency which is scheduled for 23 November 2000.

The Committee finalised a discussion paper outlining the principles to be taken into account when evaluating the plausibility of a 'condition' proposed in an application for designation. This document will be transmitted to the European Commission for incorporation into a Commission Guideline.

The Committee welcomed Dr. Marlene Haffner, Director of the Office for Orphan Product Development (OOPD) at the FDA. Dr. Haffner presented the activities of the OOPD and held an in-depth discussion with the Committee highlighting many aspects of FDA's 17 years of experience with orphan drugs.

The first oral explanations took place on 26 October 2000, with two sponsors presenting additional information or clarification to the Committee on pending applications for designation. Six positive opinions on the designation of orphan medicinal products, were adopted by the Committee, for the following conditions:

- Conditioning treatment prior to hematopoietic progenitor cell transplantation
- Erythema nodosum leprosum (ENL) or type II lepra reactions
- Essential thrombocythaemia
- Huntington's disease
- Primary and certain forms of secondary pulmonary hypertension
- Tyrosinaemia type 1

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

The COMP noted that 5 decisions on orphan designation have been granted by the European Commission[1] since the last COMP in September 2000, see Annex II.

The status of orphan designation procedures, as of 27 October 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	29	56	14	-	8

The next meeting of the COMP will be held on 21-22 November 2000.

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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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Members of the Committee for Orphan Medicinal Products 2000-2003

Chairman - Prof. Josep TORRENT-FARNELL (España)
Vice-Chairman - Mr. Yann LE CAM (Representative of Patient Organisation)

België/Belgique/Belgien	Dr. André LHOIR
Danmark	Dr. Jan RENNEBERG
Deutschland	Dr. Rembert ELBERS
Ελλάδα	Dr. George STATHOPOULOS
France	Dr. François MEYER
Ireland	Dr. Brendan BUCKLEY
Italia	Dr. Domenica TARUSCIO
Luxembourg/Luxemburg	Mme. Mariette BACKES-LIES
Nederland	Dr. H.J.J. SEEVERENS
Österreich	Prof.Dr. Hans Georg EICHLER
Portugal	Dr. José Manuel Gião TOSCANO RICO
Suomi/Finland	Dr. Kalle HOPPU
Sverige	Dr. Kerstin WESTERMARK
United Kingdom	Dr. Rashmi SHAH
EMEA Representative	Prof. Jean-Michel ALEXANDRE
EMEA Representative	*(Replacement for Dr. Mary Teeling to be nominated)*
EMEA Representative	Prof. Gianmartino BENZI
Representative of Patient Organisation	Mr. Abascal ALONSO
Representative of Patient Organisation	Mr. Alastair KENT

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

Active substance	Fluorouracil
Sponsor	Ethypharm S.A.
Orphan Indication	Treatment of glioblastoma
Opinion receipt date	18/09/00
Date of Commission Decision	18/10/00

Active substance	Gemtuzumab ozogamicin
Sponsor	Wyeth Europa Ltd.
Orphan Indication	Treatment of acute myeloid leukaemia
Opinion receipt date	18/09/00
Date of Commission Decision	18/10/00

Active substance	1,5 - (Butylimino) - 1,5 - dideoxy, D - glucitol
Sponsor	Oxford Glycosciences (UK) Ltd.
Orphan Indication	Treatment of Gaucher disease
Opinion receipt date	18/09/00
Date of Commission Decision	18/10/00

Active substance	N - Carbamyl - L - glutamic acid
Sponsor	Orphan Europe
Orphan Indication	Treatment of N - acetylglutamate synthetase (NAGS) deficiency
Opinion receipt date	18/09/00
Date of Commission Decision	18/10/00

Active substance	Arsenic trioxide
Sponsor	Voisin Consulting S.A.R.L.
Orphan Indication	Treatment of acute promyelocytic leukaemia
Opinion receipt date	18/09/00
Date of Commission Decision	18/10/00