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

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PRESS RELEASE
37th Meeting of the Committee for Orphan Medicinal Products

The thirty-seventh meeting of the Committee for Orphan Medicinal Products (COMP) took place on 29-30 July 2003.

During the meeting, The 6th Framework Programme was presented by Dr. Fergal Donnelly and Ms. Catherine Berens, DG Research, European Commission.

Following the adoption of the Orphan Regulations by EFTA countries (Norway, Iceland and Liechtenstein) as of July 2003 these three States are now fully associated with the work of the COMP. They will be non-voting COMP members. Their position on the COMP opinion will be recorded and they may act as co-ordinator for Orphan applications.

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

- Hepatocellular carcinoma
- Hodgkin lymphoma
- Cocaine poisoning
- Hyperphenylalaninemia
- Neovascular glaucoma
- Retinopathy of prematurity

Of these six opinions, four were adopted on day 47 of the procedure, well before the 90-day deadline for the Committee's opinion following receipt of a valid application.

Two negative opinions on orphan medicinal product designation were adopted. These opinions will be forwarded to the sponsors, who may submit detailed grounds for appeal within 90 days of receipt of the opinion. Three applications for which appeal procedures were ongoing were withdrawn by sponsors.

Seven oral explanations took place during the meeting. The COMP noted that sponsors withdrew four applications for orphan medicinal product designation. Fourteen decisions were granted by the European Commission on orphan designation[1] since the last COMP meeting on 12-13 June 2003, see Annex I. The status of orphan designation procedures, from January 2003 until 30 July 2003, is summarised below:

Intent to file notified	*Applications submitted*	*Positive COMP Opinions*	*Applications withdrawn*	*Appeals ongoing*	*Final negative COMP Opinions*	*Designations granted by Commission*
72	57	34	24	0	0	29

[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)

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Overview of orphan designation procedures

Year	*Applications submitted*	*Positive COMP Opinions*	*Applications withdrawn*	*Final negative COMP Opinions*	*Designations granted by Commission*
2000	72	26	3	0	14
2001	83	64	25	1	64
2002	80	43	24	3	49

Further information on designated orphan medicinal products is publicly available in the form of summarised COMP Opinions[2], which the Agency routinely publishes following adoption of the respective decisions on orphan designation by the European Commission.

The next COMP meeting will be held from 8 to 10 September 2003.

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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[2] Summarised COMP Opinions are available on the EMEA web-site.

Medicinal products Designated as Orphan Medicinal Products since the June 2003COMP Meeting

Active substance	Aldesleukin (inhalation use)
Sponsor	Chiron B.V.
Orphan Indication	Treatment of renal cell carcinoma
Opinion receipt date	16/05/2003
Date of Commission Decision	30/06/2003

Active substance	Amiloride hydrochloride dihydrate
Sponsor	Pulmo Tec GmbH
Orphan Indication	Treatment of cystic fibrosis
Opinion receipt date	16/05/2003
Date of Commission Decision	30/06/2003

Active substance	Chimeric-anti-interleukin-6 monoclonal antibody
Sponsor	Centocor B.V.
Orphan Indication	Treatment of renal cell carcinoma
Opinion receipt date	16/05/2003
Date of Commission Decision	30/06/2003

Active substance	Cytochrome P450 isoform 2B1 gene transfected human embryonic kidney 293 cells encapsulated in polymeric cellulose sulphate
Sponsor	FSG Biotechnologie Austrianova GmbH
Orphan Indication	Treatment of pancreatic cancer in combination with ifosfamide.
Opinion receipt date	16/05/2003
Date of Commission Decision	30/06/2003

Active substance	Engineered protein inhibitor of human neutrophil elastase
Sponsor	Debioclinic SA
Orphan Indication	Treatment of cystic fibrosis
Opinion receipt date	23/06/2003
Date of Commission Decision	09/07/2003

Active substance	Herpes simplex virus lacking infected cell protein 34.5
Sponsor	Crusade Laboratories Limited
Orphan Indication	Treatment of glioma
Opinion receipt date	23/06/2003
Date of Commission Decision	09/07/2003

Active substance	Hydroxyurea
Sponsor	OTL Pharma
Orphan Indication	Treatment of sickle cell syndrome
Opinion receipt date	23/06/2003
Date of Commission Decision	09/07/2003

Active substance	Murine anti-idiotypic antibody against OC125 antibody against CA125 antigen
Sponsor	Cell Control Biomedical Laboratories AG
Orphan Indication	Treatment of ovarian cancer
Opinion receipt date	23/06/2003
Date of Commission Decision	09/07/2003

Active substance	Recombinant dog gastric lipase
Sponsor	Meristem Therapeutics S.A.
Orphan Indication	Treatment of cystic fibrosis
Opinion receipt date	23/06/2003
Date of Commission Decision	09/07/2003

Active substance	Recombinant Human Arylsulfatase A
Sponsor	HemeBiotech A/S
Orphan Indication	Treatment of metachromatic leukodystrophy
Opinion receipt date	23/06/2003
Date of Commission Decision	09/07/2003

Active substance	Recombinant human insulin-like growth factor-I/recombinant human insulin-like growth factor binding protein-3
Sponsor	Insmed Incorporated
Orphan Indication	Treatment of primary growth hormone insensitivity syndrome (Laron Syndrome)
Opinion receipt date	23/06/2003
Date of Commission Decision	09/07/2003

Active substance	Aplidine
Sponsor	Pharma Mar S.A. Sociedad Unipersonal
Orphan Indication	Treatment of acute lymphoblastic leukaemia
Opinion receipt date	23/06/2003
Date of Commission Decision	09/07/2003

Active substance	Adenovirus-Interferon gamma –coding DNA sequence
Sponsor	Transgene S.A.
Orphan Indication	Treatment of Cutaneous T Cell Lymphoma
Opinion receipt date	23/06/2003
Date of Commission Decision	09/07/2003

Active substance	Prasterone
Sponsor	Medicom Healthcare BV
Orphan Indication	Treatment of Adrenal Insufficiency.
Opinion receipt date	23/06/2003
Date of Commission Decision	28/07/2003