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

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COMP/243/01

PRESS RELEASE

14th Meeting of the Committee for Orphan Medicinal Products

The Committee for Orphan Medicinal Products (COMP) held its fourteenth meeting on 11-12 June 2001.

Two oral explanations took place during the meeting, one in the framework of an appeal procedure. Following review of the grounds for appeal and the oral explanation of the sponsor, the COMP considered that the negative opinion it adopted in February 2001 should be revised and a final positive opinion on orphan designation was issued.

In total, the Committee adopted three positive opinions on the designation of orphan medicinal products, for the following conditions:

- Hepatocellular carcinoma
- Systemic secondary amyloidosis
- Wilson's disease

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

One application for orphan medicinal product designation was withdrawn by the sponsor. Six decisions on orphan designation were granted by the European Commission[1] since the last COMP meeting on 22-23 May 2001, see Annex I.

The status of orphan designation procedures, as of 12 June 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Intent to file notified	*Applications submitted*	*Applications withdrawn*	*Positive COMP Opinions*	*Negative COMP Opinions*	*Designations granted by Commission*
60	103	16	52	1[2]	43

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

The requirement for sponsors to submit annual reports to the Agency on the state of development of designated medicinal products, in accordance with Article 5(10) or Regulation (EC) No 141/2000, was discussed by the Committee. Guidance on the format and content of annual reports is under preparation, and once finalised by the Committee, will be released for consultation.

The next COMP meeting will be held on 17-18 July 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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[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/register/orphreg.htm)

[2] Of the three negative opinions reported in the May COMP Press Release one appeal is ongoing. One application has been subsequently withdrawn and one has resulted in a final positive opinion following appeal.

Medicinal products Designated as Orphan Medicinal Products on 30 May 2001

Active substance	Fomepizole
Sponsor	IDIS Ltd
Orphan Indication	Treatment of methanol poisoning
Opinion receipt date	11/4/01
Date of Commission Decision	30/5/01

Active substance	Human engineered monoclonal antibody specific for Transforming Growth Factor β2
Sponsor	Cambridge Antibody Technology Limited
Orphan Indication	Prevention of scarring in glaucoma filtration surgical procedures
Opinion receipt date	11/4/01
Date of Commission Decision	30/5/01

Active substance	Retroviral γc cDNA containing vector
Sponsor	Génopoïétic S.A.
Orphan Indication	Treatment of Severe Combined Immunodeficiency (SCID)-Xl Disease
Opinion receipt date	11/4/01
Date of Commission Decision	30/5/01

Active substance	Human Milk Fat Globule 1 / Human Milk Fat Globule 1 - S-p-isothiocyanatobenzyl-diethylenetriaminepentaacetic acid for use with 90Yttrium
Sponsor	Antisoma plc
Orphan Indication	Treatment of ovarian cancer
Opinion receipt date	11/4/01
Date of Commission Decision	30/5/01

Active substance	Ecteinascidin 743
Sponsor	Pharma Mar SA
Orphan Indication	Treatment of soft tissue sarcoma
Opinion receipt date	11/4/01
Date of Commission Decision	30/5/01

Active substance	Recombinant human alpha-1-antitrypsin (respiratory use)
Sponsor	Bayer AG
Orphan Indication	Treatment of emphysema secondary to congenital alpha 1-antitrypsin deficiency
Opinion receipt date	11/4/01
Date of Commission Decision	30/5/01