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PRESS RELEASE
Committee for Orphan Medicinal Products
January 2004 Meeting

The forty-second meeting of the Committee for Orphan Medicinal Products (COMP) took place on 13-14 January 2004.

Three 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 July 2003 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:

- Hepatitis B re-infection following liver transplantation
- Moderate and severe traumatic brain injury
- Conditioning treatment prior to haematopoietic progenitor cell transplantation

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.

The European Commission granted nine decisions on orphan designation¹ since the last COMP meeting on 4-5 December 2003 (see Annex 1).

The status of orphan designation procedures, to date for 2004, is summarised below:

<i>Applications submitted</i>	<i>Positive COMP Opinions</i>	<i>Applications withdrawn</i>	<i>Appeals ongoing</i>	<i>Final negative COMP Opinions</i>	<i>Designations granted by Commission</i>
6	3	1	-	-	1

An overview of orphan designation procedures for 2000-2003 is provided in Annex 2.

Further information on designated orphan medicinal products is publicly available in the form of summarised COMP Opinions², which the Agency routinely publishes following adoption of the respective decisions on orphan designation by the European Commission.

The Committee discussed and endorsed an update of the Commission guideline (ENTR/6283/00 Rev 2)³ on the format and content of applications for designation as orphan medicinal products. The guideline has been revised to include Icelandic and Norwegian participation in the designation process and make reference to the recent Commission Communication (2003/C 178/02)⁴. In addition, some minor editorial changes have been incorporated. When preparing an application for orphan medicinal product designation, sponsors are requested to follow this guideline.

The next COMP meeting will be held on 4-5 February 2004.

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 These documents are available on the EMEA web-site.

³ ENTR/6283/00 is available on the EMEA and Commission (<http://pharmacos.eudra.org/F2/>) web-sites

⁴ Commission Communication is published in the Official Journal of the European Union (C 178, 29.07.2003, p. 2) and is available on the Commission web-site (<http://pharmacos.eudra.org/F2/>)

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
since the December 2003 COMP Meeting**

Active substance	Recombinant antibody derivative against human CD19 and CD3
Sponsor	Micromet AG
Orphan Indication	Treatment of chronic lymphocytic leukaemia
Opinion receipt date	21/10/2003
Date of Commission Decision	1/12/2003

Active substance	Sildenafil citrate
Sponsor	Pfizer Limited
Orphan Indication	Treatment of pulmonary arterial hypertension and chronic thromboembolic pulmonary hypertension
Opinion receipt date	17/11/2003
Date of Commission Decision	12/12/2003

Active substance	3-(4'-aminoisindoline-1'-one)-1-piperidine-2,6-dione
Sponsor	Gregory Fryer Associates Ltd.
Orphan Indication	Treatment of multiple myeloma
Opinion receipt date	17/11/2003
Date of Commission Decision	12/12/2003

Active substance	4,5-dihydro-2-(2,4-dihydroxyphenyl)-4-methylthiazole-4(S)-carboxylic acid
Sponsor	Genzyme Europe B.V.
Orphan Indication	Treatment of chronic iron overload requiring chelation therapy
Opinion receipt date	17/11/2003
Date of Commission Decision	12/12/2003

Active substance	Recombinant human factor XIII (composed of two A subunits)
Sponsor	Chiltern International Limited
Orphan Indication	Treatment of hereditary factor XIII deficiency
Opinion receipt date	17/11/2003
Date of Commission Decision	12/12/2003

Active substance	5-methyl-pyridine-2-sulfonic acid {6-(2-hydroxy-ethoxy)-5-(2-methoxy-phenoxy)-2-[2-(1H-tetrazol-5-yl)-pyridin-4-yl]-pyrimidin-4-yl}-amide sodium salt
Sponsor	Axovan Europe Ltd.
Orphan Indication	Treatment of aneurysmal subarachnoid haemorrhage
Opinion receipt date	17/11/2003
Date of Commission Decision	12/12/2003

Active substance	N-acetylsarcosyl-glycyl-L-valyl-Dallo-isoleucyl-L-threonyl-L-norvalyl-L-isoleucyl-L-arginyl-L-prolyl-N-ethylamide
Sponsor	Abbott Laboratories Ltd.
Orphan Indication	Treatment of soft tissue sarcoma
Opinion receipt date	17/11/2003
Date of Commission Decision	12/12/2003

Active substance	Vasoactive Intestinal Peptide
Sponsor	Mondobiotec Laboratories Anstalt
Orphan Indication	Treatment of pulmonary arterial hypertension and chronic thromboembolic pulmonary hypertension
Opinion receipt date	24/11/2003
Date of Commission Decision	22/12/2003

Active substance	Temocillin sodium
Sponsor	Belpharma N.V.
Orphan Indication	Treatment of Burkholderia cepacia lung infection in cystic fibrosis
Opinion receipt date	12/12/2003
Date of Commission Decision	14/1/2004

**Overview of Procedures for Orphan Medicinal Product Designation
for 2000-2003**

<i>Year</i>	<i>Applications submitted</i>	<i>Positive COMP Opinions</i>	<i>Applications withdrawn</i>	<i>Final negative COMP Opinions</i>	<i>Designations granted by Commission</i>
2003	87	54	35	1	55
2002	80	43	24	3	49
2001	83	64	25	1	64
2000	72	26	3	0	14