European Medicines Agency
Pre-authorisation Evaluation of Medicines for Human Use

7 February 2005
EMEA/COMP/44905/2005

PRESS RELEASE
Committee for Orphan Medicinal Products
February 2005 Meeting

The fifty-fourth meeting of the Committee for Orphan Medicinal Products (COMP) took place on 2 February 2005.

COMP Opinions

The Committee adopted 6 positive opinions on orphan medicinal product designation during this meeting:

- (Z)-N-[2-(Diethylamino)ethyl]-5-[(5-fluoro-2-oxo-1,2-dihydro-3H-indol-3-ylidene)methyl]-2,4-dimethyl-1H-pyrrole-3-carboxamide (S)-2-hydroxysyccinate, from Pfizer Limited, for treatment of **malignant gastrointestinal stromal tumours** (review time: 60 days).
- (Z)-N-[2-(Diethylamino)ethyl]-5-[(5-fluoro-2-oxo-1,2-dihydro-3H-indol-3-ylidene)methyl]-2,4-dimethyl-1H-pyrrole-3-carboxamide (S)-2-hydroxysyccinate, from Pfizer Limited, for treatment of **renal cell carcinoma** (review time: 60 days).
- Ciclosporin (inhalation use), from Chiron Corporation Limited, for prevention of **graft rejection after lung transplantation** (review time: 60 days).
- Ciclosporin (inhalation use), from Chiron Corporation Limited, for treatment of **graft rejection after lung transplantation** (review time: 60 days).
- Cholest-4-en-3-one, oxime, from Trophos SA, for treatment of **5q spinal muscular atrophies** (review time: 88 days).
- Tipifarnib, from Janssen-Cilag International NV, for treatment of **acute myeloid leukaemia** (review time: 60 days).

One oral explanation took place during the meeting. The COMP noted that one application for orphan medicinal product designation was withdrawn by a sponsor.

Overview of orphan designation procedures

The European Commission granted five positive decisions on orphan designation[1] since the last COMP meeting on 13 January 2005 (see Annex 1).

The status of orphan designation procedures, to date for 2005, is summarised below:

Applications submitted	*Positive COMP Opinions*	*Applications withdrawn*	*Appeals ongoing*	*Final negative COMP Opinions*	*Designations granted by Commission*
10	9	2	1	-	5

An overview of orphan designation procedures for 2000-2004 is provided in Annex 2.

[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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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.

Date of next COMP meeting

The next COMP meeting will be held on 2-3 March 2005 and will be followed by an EMEA Workshop with Patient Representatives and Learned Societies on 4 March 2005.

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] These documents are available on the EMEA web-site.

Medicinal products Designated as Orphan Medicinal Products since the February 2005 COMP Meeting

Active substance	Recombinant human α -Mannosidase
Sponsor	HemeBiotech A/S
Orphan Indication	Treatment of α-Mannosidosis
Opinion receipt date	22/12/2004
Date of Commission Decision	26/1/2005

Active substance	17-allylamino-17-demethoxygeldanamycin
Sponsor	Wainwright Associates Limited
Orphan Indication	Treatment of chronic myeloid leukemia
Opinion receipt date	22/12/2004
Date of Commission Decision	26/1/2005

Active substance	Acetylcysteine
Sponsor	Zambon Group Spa
Orphan Indication	Treatment of idiopathic pulmonary fibrosis
Opinion receipt date	22/12/2004
Date of Commission Decision	26/1/2005

Active substance	L-Asparaginase
Sponsor	Medac Gesellschaft für klinische Spezialpräparate mbH
Orphan Indication	Treatment of acute lymphoblastic leukaemia
Opinion receipt date	22/12/2004
Date of Commission Decision	26/1/2005

Active substance	Recombinant human bile salt-stimulated lipase
Sponsor	Arexis AB
Orphan Indication	Treatment of cystic fibrosis
Opinion receipt date	22/12/2004
Date of Commission Decision	26/1/2005

Overview of Procedures for Orphan Medicinal Product Designation for 2000-2004

Year	*Applications submitted*	*Positive COMP Opinions*	*Applications withdrawn*	*Final negative COMP Opinions*	*Designations granted by Commission*
2004	108	75	22	3[3]	72
2003	87	54	41	1	55
2002	80	43	30	3	49
2001	83	64	27	1	64
2000	72	26	6	0	14

[3] Updated in February 2005, to include two negative Opinions for which pending appeals were cancelled.