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

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PRESS RELEASE
Committee for Orphan Medicinal Products
December 2005 Meeting

The sixty-third meeting of the Committee for Orphan Medicinal Products (COMP) took place on 7 December 2005.

The Committee welcomed Dr. Andrew Borg from the Maltese Department of Primary Health Care as a COMP member replacing Mr. Joseph Giglio.

COMP Opinions

The Committee adopted 5 positive opinions on orphan medicinal product designation during this meeting:

- *E. Coli* heat-shock protein 70 with bovine retinal S-antigen, from Biotech Tools SA, for treatment of **autoimmune uveitis** (review time: 52 days).
- Human monoclonal antibody against HLA-DR, from GPC Biotech AG, for treatment of **chronic lymphocytic leukaemia** (review time: 52 days).
- Purified inactivated Japanese encephalitis SA14-4-2 virus vaccine, from Intercell AG, for prevention of **Japanese encephalitis** (review time: 52 days).
- Lentiviral vector containing the human Wiskott Aldrich syndrome protein gene, from Généthon, for treatment of **Wiskott Aldrich syndrome** (review time: 52 days).
- Vandetanib, from AstraZeneca UK Limited, for treatment of **medullary thyroid carcinoma** (review time: 52 days).

The COMP noted that two applications for orphan medicinal product designation were withdrawn by sponsors, one during the evaluation phase and one during the validation phase of the procedure.

Further to the COMP opinion of September 2005 recommending the designation of recombinant modified vaccinia virus Ankara expressing tuberculosis antigen 85A, a booster vaccine for prevention of tuberculosis disease, on the grounds that marketing of the medicinal product would be unlikely to generate sufficient return to justify the necessary investment, the Committee received several questions on the possible consequences of this opinion. Further clarification has been published on the EMEA website.

Overview of orphan designation procedures

Four positive decisions on orphan designation[1] have been received from the European Commission since the last COMP meeting on 9-10 November 2005 (see Annex 1).

[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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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*
105	88	27	-	-	75

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

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.

Applications for Marketing Authorisation for Orphan Medicinal Products

No designated orphan medicinal products have been the subject of a centralised application for marketing authorisation since the last COMP meeting.

COMP Guideline on Elements Required to Support the Medical Plausibility and the Assumption of Significant Benefit for an Orphan Designation

Following public consultation, the COMP has finalised the Guideline on Elements Required to Support the Medical Plausibility and the Assumption of Significant Benefit for an Orphan Designation (EMEA/COMP/66972/2004). An overview of the comments received on the draft guideline together with the outcome of its discussion by the Committee will be published on the EMEA website.

Date of next COMP meeting

The next COMP meeting will be held on 10-11 January 2006.

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.

Orphan Medicinal Product Designations received since the November 2005 COMP Meeting

Active substance	Human *Staphylococcus aureus* immunoglobulin
Sponsor	Nabi Biopharmaceuticals Europe Ltd
Orphan Indication	Treatment of *Staphylococcus aureus* bacteraemia
Opinion receipt date	22/09/2005
Date of Commission Decision	03/11/2005

Active substance	Imatinib mesilate
Sponsor	Novartis Europharm Limited
Orphan Indication	Treatment of chronic eosinophilic leukaemia and the hypereosinophilic syndrome
Opinion receipt date	22/09/2005
Date of Commission Decision	28/10/2005

Active substance	Recombinant modified vaccinia virus Ankara expressing tuberculosis antigen 85A
Sponsor	University of Oxford
Orphan Indication	Prevention of tuberculosis disease in BCG vaccinated individuals
Opinion receipt date	22/09/2005
Date of Commission Decision	28/10/2005

Active substance	Peptide 144 TGF-beta1-inhibitor (TSLDASIIWAMMQN)
Sponsor	Digna Biotech S.L.
Orphan Indication	Treatment of systemic sclerosis
Opinion receipt date	22/09/2005
Date of Commission Decision	28/10/2005

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	4	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