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

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PRESS RELEASE
Committee for Orphan Medicinal Products
February 2007 Meeting

The seventy-sixth meeting of the Committee for Orphan Medicinal Products (COMP) took place on 6-7 February 2007. The Committee was delighted to officially welcome Prof. Björn Beerman as the new COMP member nominated by Sweden.

COMP Opinions

The Committee adopted 7 positive opinions on orphan medicinal product designation during this meeting:

- Elafin, from Proteo Biotech AG, **for treatment of pulmonary arterial hypertension and chronic thromboembolic pulmonary hypertension** (review time: day 90)
- Enzastaurin hydrochloride, from Eli Lilly Nederland B.V., **for treatment of diffuse large B cell Lymphoma** (review time: day 59)
- Hydrocortisone (modified release tablet), from Phoqus Pharmaceuticals Limited, **for treatment of adrenal insufficiency** (review time: day 59)
- Idebenone, from Santhera Pharmaceuticals (Deutschland) AG, **for treatment of Duchenne muscular dystrophy** (review time: day 90)
- Recombinant adeno-associated viral vector containing human alpha-1 antitrypsin gene, from The Matthews Consultancy Ltd, **for treatment of congenital alpha-1 antitrypsin deficiency** (review time: day 59)
- Recombinant human monoclonal antibody to human IL-1beta of the IgG1/K class, from Novartis Europharm Limited, **for treatment of cryopirin-associated periodic syndromes (Familial Cold Urticaria Syndrome (FCUS), Muckle-Wells Syndrome (MWS), and Neonatal Onset Multisystem Inflammatory Disease (NOMID), also known as Chronic Infantile Neurological Cutaneous Articular Syndrome (CINCA))** (review time: day 59)
- Zanolimumab, from Serono Europe Limited, **for treatment of peripheral T-cell lymphoma (nodal, other extranodal and leukaemic/disseminated)** (review time: day 59)

Two oral explanations on orphan medicinal product designation took place during the meeting.

Withdrawals of Orphan Medicinal Product Applications

The COMP noted that no applications for orphan medicinal product designation were withdrawn.

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Overview of orphan designation procedures

The European Commission granted five positive decisions on orphan designation[1] since the last COMP meeting on 9-10 January 2007 (see Annex 1).

The status of orphan designation procedures, to date for 2007, is summarised below:

Applications submitted	*Positive COMP Opinions*	*Applications withdrawn*	*Appeals ongoing*	*Final negative COMP Opinions*	*Designations granted by Commission*
-	13	3	-	-	5

An overview of orphan designation procedures for 2000-2006 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.

Date of next COMP meeting

The next COMP meeting will be held on 7-8 March 2007.

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.europa.eu.

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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://ec.europa.eu/enterprise/pharmaceuticals/index_en.htm)

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

Orphan Medicinal Product Designations received since the January 2007 COMP Meeting

Active substance	Complement factor H
Sponsor	Laboratoire français du Fractionnement et des Biotechnologies (LFB)
Orphan Indication	Treatment of atypical Haemolytic Uraemic Syndrome (aHUS) associated with an inherited abnormality of the complement system
Opinion receipt date	21 December 2006
Date of Commission Decision	26 January 2007

Active substance	Fenretinide
Sponsor	Cancer Research UK
Orphan Indication	Treatment of primary malignant bone tumours
Opinion receipt date	21 December 2006
Date of Commission Decision	26 January 2007

Active substance	Forodesine hydrochloride
Sponsor	Napp Pharmaceuticals Research Limited
Orphan Indication	Treatment of cutaneous T-cell lymphoma
Opinion receipt date	21 December 2006
Date of Commission Decision	29 January 2007

Active substance	Recombinant modified vaccinia Ankara expressing human 5T4
Sponsor	Oxford Biomedica (UK) Ltd
Orphan Indication	Treatment of renal cell carcinoma
Opinion receipt date	21 December 2006
Date of Commission Decision	26 January 2007

Active substance	Thiotepa
Sponsor	Adienne S.r.l.
Orphan Indication	Conditioning treatment prior to haematopoietic progenitor cell transplantation
Opinion receipt date	21 December 2006
Date of Commission Decision	29 January 2007

Overview of Procedures for Orphan Medicinal Product Designation for 2000-2006

Year	*Applications submitted*	*Positive COMP Opinions*	*Applications withdrawn*	*Final negative COMP Opinions*	*Designations granted by Commission*
2006	104	81	20	2	80
2005	118	88	30	0	88
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