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

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PRESS RELEASE
Committee for Orphan Medicinal Products
May 2007 Meeting

The seventy-ninth meeting of the Committee for Orphan Medicinal Products (COMP) took place on 30-31 May 2007.

COMP Opinions for Orphan Medicinal Product Designation

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

- 1-{3-[3-(4-chlorophenyl)propoxy]propyl}piperidine, hydrochloride, from Bioprojet, **for treatment of narcolepsy** (review time: day 89)
- Fampridine, from Dr Ulrich Granzer, **for treatment of Guillain-Barré syndrome** (review time: day 89)
- Lusupultide, from ALTANA Pharma AG, **for treatment of aspiration pneumonitis requiring intubation and mechanical ventilation** (review time: day 89)
- Recombinant adeno-associated viral vector containing human acid alfa-glucosidase-gene, from The Matthews Consultancy Ltd, **for treatment of Pompe Disease** (review time: day 89)
- Rilonacept, Regeneron UK 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 63)

Withdrawals of Orphan Medicinal Product Applications

The COMP noted that three applications for orphan medicinal product designation were withdrawn during evaluation.

Overview of orphan designation procedures

The European Commission granted 4 positive decisions on orphan designation[1] since the last COMP meeting on 11-12 April 2007 (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://ec.europa.eu/enterprise/pharmaceuticals/index_en.htm)

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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Applications submitted	*Positive COMP Opinions*	*Applications withdrawn*	*Appeals ongoing*	*Final negative COMP Opinions*	*Designations granted by Commission*
53	29	9	-	-	24

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 26-28 June 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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[2] These documents are available on the EMEA web-site.

Orphan Medicinal Product Designations received since the April 2007 COMP Meeting

Active substance	Antisense Oligonucleotide (TATCCGGAGGGCTCGCCATGCTGCT)
Sponsor	Gene Signal SAS
Orphan Indication	Prevention of corneal graft rejection
Opinion receipt date	21 March 2007
Date of Commission Decision	17 April 2007

Active substance	Autologous CD34+ cells transfected with lentiviral vector containing the human arylsulfatase A cDNA
Sponsor	Fondazione Telethon
Orphan Indication	Treatment of metachromatic leukodystrophy
Opinion receipt date	22 March 2007
Date of Commission Decision	13 April 2007

Active substance	Nilotinib hydrochloride monohydrate
Sponsor	Novartis Europharm Limited
Orphan Indication	Treatment of gastro intestinal stromal tumours
Opinion receipt date	22 March 2007
Date of Commission Decision	13 April 2007

Active substance	Pralatrexate
Sponsor	Oxford Regulatory Solutions Ltd
Orphan Indication	Treatment of peripheral T-cell lymphoma (nodal, other extranodal and leukaemic/disseminated)
Opinion receipt date	22 March 2007
Date of Commission Decision	13 April 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