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

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COMMITTEE FOR ORPHAN MEDICINAL PRODUCTS
SEPTEMBER 2009 PLENARY MEETING
MONTHLY REPORT

The Committee for Orphan Medicinal Products (COMP) held its 104th plenary meeting on 1-2 September 2009.

During this meeting, the members of the Committee elected the new Chairperson and the Vice-Chairperson for a term of three years. Dr Kerstin Westermark (Sweden) was elected as Chairperson, and Mrs. Birthe Byskov Holm (Patient Representative nominated by the European Commission) was elected as Vice-Chairperson. The Committee and the European Medicines Agency congratulated the new Chairperson and Vice-Chairperson.

ORPHAN MEDICINAL PRODUCT DESIGNATION

The COMP adopted 13 positive opinions recommending the following medicines for designation as orphan medicinal products to the European Commission:

For the following medicines the EMEA review began on 5 June 2009 with an active review time of 90 days.

- **Patupilone** for treatment of primary peritoneal cancer, Novartis Europharm Limited.
- **Patupilone** for treatment of fallopian tube cancer, Novartis Europharm Limited.

For the following medicines the EMEA review began on 10 July 2009 with an active review time of 55 days.

- **4-benzyl-2-naphtalen-1-yl-1,2,4-thiadiazolidine-3,5-dione** for treatment of progressive supranuclear palsy, NOSCIRA, S.A.
- **5'-O-(*trans*-9''-octadecenoyl)-1-beta-D-2'deoxy-2',2'-difluorocytidine** for treatment of pancreatic cancer, Clavis Pharma ASA.
- **6-chloro-2,3,4,9-tetrahydro-1*H*-carbazole-1-carboxamide** for treatment of Huntington's disease, Sienna Biotech SpA.
- **Anti-EphA2 monoclonal antibody conjugated to maleimidocaproyl monomethylauristatin phenylalanine** for treatment of ovarian cancer, MedImmune Ltd.
- **Cholic acid** for treatment of inborn errors in primary bile acid synthesis responsive to treatment with cholic acid, Special Products Ltd.
- **Human anthrax immunoglobulin** for treatment on inhalation anthrax disease, Emergent Sales and Marketing Germany GmbH.
- **Human anthrax immunoglobulin** for post-exposure prophylaxis of inhalation anthrax disease, Emergent Sales and Marketing Germany GmbH.
- **Masitinib mesilate** for treatment of pancreatic cancer, AB Science.
- **N-[(2S)-2,3-dihydroxypropyl]-3-[(2-fluoro-4-iodophenyl)amino]isonicotinamide hydrochloride** for treatment of pancreatic cancer, Merck KGaA.

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- **NGR-human tumour necrosis factor** for treatment of hepatocellular carcinoma, MolMed S.p.A.
- **Peptides mimicking antigen receptors on autoimmune B cells and autoimmune T cells associated with myasthenia gravis** for treatment of myasthenia gravis, CuraVac Europe sprl.

Public summaries of opinion will be available on the EMEA website which the Agency updates following adoption of the respective decisions on orphan designation by the European Commission.

OTHER INFORMATION ON THE ORPHAN MEDICINAL PRODUCT DESIGNATION

Lists of questions

The COMP adopted six lists of questions on initial applications. These applications will be discussed again at the next COMP plenary meeting prior to adoption of the opinion.

Oral hearings

Seven oral hearings took place.

Withdrawals of applications for orphan medicinal product designation

The COMP noted that four applications for orphan medicinal product designation were withdrawn.

Detailed information on the orphan designation procedure

An overview of orphan designation procedures since 2000 is provided in **Annex 1**.

The list of medicinal products for which decisions on orphan designation[1] have been given by the European Commission since the last COMP plenary meeting is provided in **Annex 2**.

Details on the opinions for marketing authorisation for orphan medicinal products adopted by the Committee for Medicinal Products for Human Use (CHMP) can be found in the CHMP Monthly Report on the EMEA website.

Article 5 (12) of Regulation (EC) No 141/2000 of the European Parliament and of the Council

In line with its responsibility to review whether or not a designated orphan medicinal product still fulfils the designation criteria prior to the granting of a marketing authorisation, the COMP adopted prior to this meeting three opinions recommending to the European Commission that the following orphan medicinal products be kept in the Community registry of orphan medicinal products via written procedure on 4 August 2009:

- **Arcalyst** (Rilonacept), from Regeneron UK Limited, for the 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))
- **Ilaris** (Recombinant human monoclonal antibody to human IL-1 beta of the IgG1/K class), from Novartis Europharm limted, for the 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))

[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)

- **Torisel** (Temsirolimus), from Wyeth Europea Limited, for the treatment of mantle cell lymphoma

UPCOMING MEETINGS FOLLOWING THE SEPTEMBER 2009 COMP PLENARY MEETING

- The Informal COMP meeting will be held on 1-2 October 2009 in Stockholm.
- The next meeting of the COMP will be held 6-7 October 2009.

ORGANISATIONAL MATTERS

The main topics addressed during the September 2009 COMP meeting related to:

- The elections of Prof. K. Westermark as the COMP Chair and Ms B. Byskov Holm as the Vice-Chair for next three-year term.
- One Protocol Assistance letter was adopted.

NOTE: This Monthly Report and other documents may be found on the internet at the following location: http://www.emea.europa.eu

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OVERVIEW FOR ORPHAN MEDICINAL PRODUCT DESIGNATION PROCEDURE SINCE 2000

Year	Applications submitted	Positive COMP Opinions	Applications withdrawn	Final negative COMP Opinions	Designations granted by Commission
2009	114	79	13	1	64
2008	119	86	31	1	73
2007	125	97	19	1	98
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

MEDICINAL PRODUCTS GRANTED A COMMUNITY DESIGNATION AS ORPHAN MEDICINAL PRODUCT SINCE THE JULY 2009 COMP PLENARY REPORT BY THE EUROPEAN COMMISSION

Active substance	(S)-3´-(OH)-desazadesferrithiocin-polyether, magnesium salt
Sponsor	FerroKin BioSciences Ltd
Orphan Indication	Treatment of chronic iron overload requiring chelation therapy
COMP Opinion date	04/06/2009
Orphan Designation date	24/07/2009

Active substance	Afamelanotide
Sponsor	Clinuvel UK Limited
Orphan Indication	Treatment of solar urticaria
COMP Opinion date	04/06/2009
Orphan Designation date	24/07/2009

Active substance	Allogeneic ex vivo expanded umbilical cord blood cells
Sponsor	Teva Pharma GmbH
Orphan Indication	Treatment of Hodgkin lymphoma
COMP Opinion date	04/06/2009
Orphan Designation date	24/07/2009

Active substance	Blinatumomab
Sponsor	Micromet AG
Orphan Indication	Treatment of acute lymphoblastic leukaemia
COMP Opinion date	04/06/2009
Orphan Designation date	24/07/2009

Active substance	Ciclosporin (eye drops, solution)
Sponsor	Allergan Pharmaceuticals Ireland
Orphan Indication	Treatment of atopic keratoconjunctivitis
COMP Opinion date	04/06/2009
Orphan Designation date	24/07/2009

Active substance	Ciprofloxacin (liposomal)
Sponsor	Interface International Consultancy Ltd
Orphan Indication	Treatment of cystic fibrosis
COMP Opinion date	04/06/2009

Orphan Designation date	24/07/2009

Active substance	Eculizumab
Sponsor	Alexion Europe SAS
Orphan Indication	Treatment of atypical haemolytic uremic syndrome
COMP Opinion date	04/06/2009
Orphan Designation date	24/07/2009

Active substance	Hypothiocyanite / lactoferrin
Sponsor	Alaxia
Orphan Indication	Treatment of cystic fibrosis
COMP Opinion date	04/06/2009
Orphan Designation date	24/07/2009

Active substance	Octocog alpha (liposomal)
Sponsor	Bayer Schering Pharma AG
Orphan Indication	Treatment of haemophilia A
COMP Opinion date	04/06/2009
Orphan Designation date	24/07/2009

Active substance	Recombinant histidine-tagged idiotype immunoglobulin Fab fragment of clonal B-cell receptors
Sponsor	CellGenix Technologie Transfer GmbH
Orphan Indication	Treatment of diffuse large B-cell lymphoma
COMP Opinion date	04/06/2009
Orphan Designation date	24/07/2009

Active substance	Recombinant human N-acetylgalactosamine-6-sulfatase
Sponsor	BioMarin Europe Limited
Orphan Indication	Treatment of mucopolysaccharidosis, type IVA (Morquio A Syndrome)
COMP Opinion date	04/06/2009
Orphan Designation date	24/07/2009

Active substance	Tamibarotene
Sponsor	Eudax S.R.L.
Orphan Indication	Treatment of acute promyelocytic leukaemia.
COMP Opinion date	04/06/2009

Orphan Designation date	24/07/2009

Active substance	Tosedostat
Sponsor	Chroma Therapeutics Ltd
Orphan Indication	Treatment of acute myeloid leukaemia
COMP Opinion date	04/06/2009
Orphan Designation date	24/07/2009

Active substance	Trabedersen
Sponsor	Antisense Pharma GmbH
Orphan Indication	Treatment of pancreatic cancer
COMP Opinion date	04/06/2009
Orphan Designation date	24/07/2009