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

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COMMITTEE FOR ORPHAN MEDICINAL PRODUCTS
JUNE 2009 PLENARY MEETING
MONTHLY REPORT

The Committee for Orphan Medicinal Products (COMP) held its 102nd plenary meeting on 3-4 June 2009. The Committee decided, by majority, to postpone the election of the Chair and the Vice-chair to the COMP meeting following the nomination of the 4 members to the COMP by the EC. The Committee agreed that until than the meetings would be chaired by Prof. K. Westermark.

End of the 3rd mandate and nominations for COMP members

The Committee thanked warmly Dr Domenica Tarucio (appointed member from Italy), for her successful contribution to the work of the Committee. The Chair welcomed Dr Tatiana Foltánová (appointed member from Slovak Republic) and Prof. Maurizio Clementi (appointed member from Italy). The Committee welcomed also Ms. Mirjam Söderholm, who will be working with COMP-EMEA activities together with Ms Claire Scharf-Kröner at the European Commission, DG Enterprise, Pharmaceuticals.

7th Framework Programme research in rare diseases

The Committee welcomed Ms Eleni Psychari, Dr Sophie Koutouzov (GIS-Institute des Maladies Rares) and Dr Brigit Wetterauer (BMBF) who presented E-rare project to the Committee. The Committee also welcomed Dr C. Berens (EC DG Research) who presented an overview of the 7th Framework Programme (FP7) 2007-2013 past and future calls in the field of rare diseases.

ORPHAN MEDICINAL PRODUCT DESIGNATION

The COMP adopted 14 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 9 March 2009 with an active review time of 88 days.

- **Hypothiocyanite and lactoferrin,** for treatment of cystic fibrosis, from Alaxia
- **(*S*)-3'-(OH)-desazadesferrithiocin-polyether, magnesium salt** for treatment of chronic iron overload requiring chelation therapy, from FerroKin BioSciences Ltd
- **Tamibarotene** for treatment of acute promyelocytic leukaemia, from Eudax S.R.L

For the following medicines the EMEA review began on 14 April 2009 with an active review time of 52 days.

- **Afamelanotide** for treatment of solar urticaria, from Clinuvel UK Limited
- **Allogeneic *ex vivo* expanded umbilical cord blood cells** for treatment of Hodgkin lymphoma, from Teva Pharma GmbH
- **Blinatumomab** for treatment of acute lymphoblastic leukaemia, from Micromet AG
- **Ciclosporin (eye drops, solution)** for treatment of atopic keratoconjunctivitis, from Allergan Pharmaceuticals Ireland

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- **Ciprofloxacin (lisosomal)** for treatment of cystic fibrosis, from Interface International Consultancy Ltd
- **Eculizumab** for treatment of atypical haemolytic uremic syndrome, from Alexion Europe SAS
- **Octocog alpha (liposomal)** for treatment of haemophilia A, from Bayer Schering Pharma AG
- **Recombinant hisitidine-tagged idiotype immunoglobulin Fab fragment of clonal B-cell receptors (IdioVax)** for treatment of diffuse large B-cell lymphoma, from CellGenix Technologie Transfer GmbH
- **Recombinant human N-acetylgalactosamine-6-sulfatase** for treatment of mucopolysaccharidosis, type IVA (Morquio A Syndrome), from BioMarin Europe Ltd
- **Tosedostat** for treatment of acute myeloid leukaemia, from Chroma Therapeutics Ltd
- **Trabedersen** for treatment of pancreatic cancer, from Antisense Pharma GmbH

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 8 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

Three oral hearings took place.

Withdrawal of application for orphan medicinal product designation

The COMP noted that one application for orphan medicinal product designation was 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**.

Applications for marketing authorisation for orphan medicinal products

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 2 opinions recommending to the European Commission that the following orphan medicinal products be kept in the Community registry of orphan medicinal products:

- **Afinitor (Everolimus)** from Novartis Europharm Ltd, for treatment of renal cell carcinoma;
- **Mozobil (l, 1'-[1,4-phenylenebis (methylene)]-bis-1,4,8,11-tetraazacyclotetradecane),** from Genzyme BV, for treatment to mobilize progenitor cells prior to stem cell transplantation.

[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)

UPCOMING MEETINGS FOLLOWING THE JUNE 2009 COMP PLENARY MEETING

- The next meeting of the COMP will be held on 7-8 July 2009.

OTHER MATTERS

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

- The appointment of Dr Tatiana Foltánová as the new member appointed from Slovak Republic.
- The appointment of Prof. Maurizio Clementi as the new member appointed from Italy.
- Discussion on the new call from DG Research.
- Two Protocol Assistance letters were 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	61	48	6	-	47
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 MAY 2009 COMP PLENARY REPORT BY THE EUROPEAN COMMISSION

Active substance	2',3',5'-tri-O-acetyluridine
Sponsor	Wellstat Therapeutics EU Limited
Orphan Indication	Treatment of 5-fluorouracil overdose
COMP Opinion date	02/04/2009
Orphan Designation date	15/05/2009

Active substance	4,6,8-trihydroxy-10-(3,7,11-trimethyldodeca-2,6,10-trienyl)-5,10-dihydrodibenzo[b,e][1,4]diazepin-11-one
Sponsor	Albany Regulatory Consulting limited
Orphan Indication	Treatment of glioma
COMP Opinion date	02/04/2009
Orphan Designation date	15/05/2009

Active substance	Adeno-associated viral vector containing porphobilinogen deaminase gene
Sponsor	Amsterdam Molecular Therapeutics BV
Orphan Indication	Treatment of acute intermittent porphyria
COMP Opinion date	04/03/2009
Orphan Designation date	29/04/2009

Active substance	Alicaforsen
Sponsor	Atlantic Healthcare Limited
Orphan Indication	Treatment of pouchitis
COMP Opinion date	02/04/2009
Orphan Designation date	15/05/2009

Active substance	Autologous haematopoietic stem cells transduced with lentiviral vector encoding the human beta-globin gene
Sponsor	EGT San Rocco Italia SRL
Orphan Indication	Treatment of beta-thalassaemia intermedia and major
COMP Opinion date	04/03/2009
Orphan Designation date	29/04/2009

Active substance	Autologous tumor-derived gp96 heat shock protein-peptide complex
Sponsor	Antigenics Therapeutics Limited

Orphan Indication	Treatment of glioma
COMP Opinion date	04/03/2009
Orphan Designation date	29/04/2009

Active substance	Dexamethasone phosphate (iontophoretic solution, ocular use)
Sponsor	Voisin Consulting S.A.R.L.
Orphan Indication	Treatment of corneal graft rejection
COMP Opinion date	02/04/2009
Orphan Designation date	15/05/2009

Active substance	Guanabenz
Sponsor	Acure Pharma AB
Orphan Indication	Treatment of traumatic spinal cord injury
COMP Opinion date	04/03/2009
Orphan Designation date	29/04/2009

Active substance	Humanised IgG4 monoclonal antibody to the human toll-like receptor type 2
Sponsor	Opsona Therapeutics
Orphan Indication	Prevention of the ischaemia/reperfusion injury associated with solid organ transplantation
COMP Opinion date	02/04/2009
Orphan Designation date	15/05/2009

Active substance	L-asparaginase encapsulated in erythrocytes
Sponsor	Erutech Pharma SA
Orphan Indication	Treatment of pancreatic cancer
COMP Opinion date	02/04/2009
Orphan Designation date	15/05/2009

Active substance	Lintuzumab
Sponsor	Seattle Genetics UK Limited
Orphan Indication	Treatment of myelodysplastic syndromes
COMP Opinion date	04/03/2009
Orphan Designation date	29/04/2009

Active substance	Lintuzumab
Sponsor	Seattle Genetics UK Limited

Orphan Indication	Treatment of acute myeloid leukaemia
COMP Opinion date	04/03/2009
Orphan Designation date	30/04/2009

Active substance	Mercaptopurine (oral suspension)
Sponsor	Nova Laboratories Limited
Orphan Indication	Treatment of acute lymphoblastic leukaemia
COMP Opinion date	04/03/2009
Orphan Designation date	30/04/2009

Active substance	Nanobody directed towards the human A1 domain of von Willebrand factor
Sponsor	Ablynx NV
Orphan Indication	Treatment of thrombotic thrombocytopenic purpura
COMP Opinion date	04/03/2009
Orphan Designation date	30/04/2009

Active substance	Pegylated recombinant human factor IX
Sponsor	Novo Nordisk A/S
Orphan Indication	Treatment of haemophilia B
COMP Opinion date	02/04/2009
Orphan Designation date	15/05/2009

Active substance	S-[2,3-bispalmitoyloxy-(2R)-propyl]-cysteinyl-GNNDESNISFKEK
Sponsor	MBiotec GmbH
Orphan Indication	Treatment of pancreatic cancer
COMP Opinion date	02/04/2009
Orphan Designation date	15/05/2009

Active substance	Skin equivalent graft genetically corrected with a COL7A1-encoding SIN retroviral vector
Sponsor	Prof. Alain Hovnanian
Orphan Indication	Treatment of dystrophic epidermolysis bullosa
COMP Opinion date	04/03/2009
Orphan Designation date	30/04/2009

Active substance	Talampanel
Sponsor	Teva Pharma GmbH

Orphan Indication	Treatment of glioma
COMP Opinion date	04/03/2009
Orphan Designation date	29/04/2009

Active substance	Treprostinil diethanolamine
Sponsor	United Therapeutics Europe Ltd
Orphan Indication	Treatment of systemic sclerosis
COMP Opinion date	02/04/2009
Orphan Designation date	15/05/2009