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

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COMMITTEE FOR ORPHAN MEDICINAL PRODUCTS
NOVEMBER 2009 PLENARY MEETING
MONTHLY REPORT

The Committee for Orphan Medicinal Products (COMP) held its 106th plenary meeting on 4-5 November 2009. The Committee designated for the first time more than 100 orphan medicinal products in one calendar year.

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 10 August 2009 with an active review time of 88 days.

- **Recombinant human elafin** for treatment of oesophagus carcinoma, from Proteo Biotech AG.
- **Givinostat** for treatment for the systemic-onset juvenile idiopathic arthritis, from Italfarmaco S.p.A.
- **Lithium citrate tetrahydrate (in reverse-micelle formulation)** for treatment of Huntington's disease, from Medesis Pharma.

For the following medicines the EMEA review began on 15 September 2009 with an active review time of 52 days.

- **2-iminobiotin** for treatment of perinatal asphyxia, from Neurophyxia B.V.
- **Beta-artemether / lumefantrine (powder for oral suspension)** for treatment of malaria, from Dafra Pharma International NV.
- **Brivudine** for treatment of pancreatic cancer, from RESprotect GmbH.
- **Human monoclonal antibody against *Pseudomonas aeruginosa* IATS-O1** for treatment of Pneumonia caused by serotype O1 *Pseudomonas aeruginosa*, from Envestia Limited.
- **Macitentan** for treatment of idiopathic pulmonary fibrosis, from Actelion Registration Limited
- **Pegylated recombinant phenylalanine ammonia lyase** for treatment of hyperphenylalaninaemia, from BioMarin Europe Ltd.
- **Recombinant fusion protein consisting of the extracellular portion of CD95 fused to the Fc part of a human IgG1 molecule** for treatment of glioma, from Apogenix.
- **Recombinant human vascular endothelial growth factor** for treatment of amyotrophic lateral sclerosis, from NeuroNova AB.
- **Recombinant kallikrein inhibitor** for treatment of Netherton Syndrome, from Voisin Consulting S.A.R.L.

* Amended number of adopted positive opinions.

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- ***Streptococus pyogenes* SU strain treated with benzylpenicillin** for treatment of congenital lymphatic malformations, from Theradex (Europe) Ltd.
- **Veltuzumab** for treatment of chronic lymphocytic leukaemia, from Immunomedics 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

List of questions

The COMP adopted one list of questions on an initial application. This application 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 five 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[†] 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 one opinion recommending to the European Commission that the following orphan medicinal product be kept in the Community registry of orphan medicinal products:

- **Zenas, (3,4 diamonipyridine phosphate),** from EUSA Pharma SAS, for treatment of Lambert-Eaton myasthenic syndrome

UPCOMING MEETINGS FOLLOWING THE NOVEMBER 2009 COMP PLENARY MEETING

- The 107th meeting of the COMP will be held on 2-3 December 2009.

[†] 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)

OTHER MATTERS

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

- One Protocol Assistance letter was adopted.
- The minutes of the Informal COMP meeting held on 1-2 October 2009 in Stockholm, Sweden were presented. It was agreed during this meeting to work further to increase transparency with stakeholders as well as to improve the coordination between the COMP and other Committees of the Agency such as the PDCO and the newly formed CAT.
- The Committee was informed that the European Commission is consulting stakeholders on how the clinical Trials Directive (Directive 2001/20/EC) might be improved under the following link http://ec.europa.eu/enterprise/pharmaceuticals/clinicaltrials/docs/2009_10_09_public-consultation-paper.pdf. Comments to the public consultation document are due by January 8th 2009.
- The Committee was presented the draft Agenda for the 10th Year Anniversary of the EU Orphan Drugs Legislation Workshop to be held on 3-4 May 2010 at the Agency.
- Furthermore regarding the clarification of treatment recommendations for the orphan medicinal product Cerezyme, as referred in the October 2009 press release for the CHMP http://www.emea.europa.eu/pdfs/human/press/pr/67119009en.pdf, the CHMP agreed to further update the temporary treatment recommendations for Cerezyme to deal with the supply shortage of the medicine. The new temporary recommendations aim to ensure that patients at greatest need continue to receive Cerezyme. The medicine is used in the treatment of patients with Gaucher disease, a disease in which patients do not have enough of an enzyme called glucocerebrosidase.

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	138	101	21	1	94
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 OCTOBER 2009 COMP PLENARY REPORT BY THE EUROPEAN COMMISSION

Active substance	(-)-trans-3-(5,6-dihydro-4H-pyrrolo[3,2,1-ij]quinolin-1yl)-4-(1H-indol-3-yl) pyrrolidine-2, 5-dione
Sponsor	Gregory Fryer Associates Ltd
Orphan Indication	Treatment of soft tissue sarcoma
COMP Opinion date	08/07/2009
Orphan Designation date	08/10/2009

Active substance	(S)-ethyl 2-amino-3-(4-(2-amino-6-((R)-1-(4-chloro-2-(3-methyl-1H-pyrazol-1-yl)phenyl)-2,2,2-trifluoroethoxy)pyrimidin-4-yl)phenyl)propanoate
Sponsor	Lexicon Celtic Limited
Orphan Indication	Treatment of carcinoid tumours
COMP Opinion date	08/07/2009
Orphan Designation date	08/10/2009

Active substance	26 base single stranded phosphodiester DNA oligonucleotide
Sponsor	Antisoma Research Ltd
Orphan Indication	Treatment of acute myeloid leukaemia
COMP Opinion date	08/07/2009
Orphan Designation date	08/10/2009

Active substance	4-benzyl-2-naphtalen-1-yl-1,2,4-thiadiazolidine-3,5-dione
Sponsor	NOSCIRA, S.A.
Orphan Indication	Treatment of progressive nuclear palsy
COMP Opinion date	02/09/2009
Orphan Designation date	28/10/2009

Active substance	5'-O-(trans-9"-octadecenoyl)-1-beta-D-2'-deoxy-2',2'-difluorocytidine
Sponsor	Clavis Pharma ASA
Orphan Indication	Treatment of pancreatic cancer
COMP Opinion date	02/09/2009
Orphan Designation date	28/10/2009

Active substance	6-chloro-2,3,4,9-tetrahydro-1H-carbazole-1-carboxamide
Sponsor	Siena Biotech SpA
Orphan Indication	Treatment of Huntington's disease
COMP Opinion date	02/09/2009
Orphan Designation date	28/10/2009

Active substance	Adeno-associated viral vector containing modified U1 snRNA
Sponsor	Amsterdam Molecular Therapeutics (AMT) B.V.
Orphan Indication	Treatment of Duchenne muscular dystrophy
COMP Opinion date	08/07/2009
Orphan Designation date	08/10/2009

Active substance	Allogeneic ex vivo expanded umbilical cord blood cells
Sponsor	Teva Pharma GmbH
Orphan Indication	Treatment of myelodysplastic syndromes
COMP Opinion date	08/07/2009
Orphan Designation date	08/10/2009

Active substance	Allogeneic ex vivo expanded umbilical cord blood cells
Sponsor	Teva Pharma GmbH
Orphan Indication	Treatment of chronic myeloid leukaemia
COMP Opinion date	08/07/2009
Orphan Designation date	08/10/2009

Active substance	Anti-EphA2 monoclonal antibody conjugated to maleimidocaproyl monomethylauristatin phenylalanine
Sponsor	MedImmune Ltd
Orphan Indication	Treatment of ovarian cancer
COMP Opinion date	02/09/2009
Orphan Designation date	28/10/2009

Active substance	Cholic acid
Sponsor	Special Products Ltd
Orphan Indication	Treatment of inborn errors of primary bile acid synthesis responsive to treatment with cholic acid
COMP Opinion date	02/09/2009
Orphan Designation date	28/10/2009

Active substance	Human C1-inhibitor
Sponsor	ViroPharma SPRL
Orphan Indication	Treatment of angioedema caused by C1-inhibitor deficiency
COMP Opinion date	08/07/2009
Orphan Designation date	08/10/2009

Active substance	Human tumour necrosis factor alfa-derived peptide Cys-Gly-Gln-Arg-Glu-Thr-Pro-Glu-Gly-Ala-Glu-Ala-Lys-Pro-Trp-Tyr-Cys
Sponsor	Apeptico forschung und Entwicklung GmbH
Orphan Indication	Treatment of acute lung injury
COMP Opinion date	08/07/2009
Orphan Designation date	08/10/2009

Active substance	Eicosapentaenoic acid
Sponsor	S.L.A. Pharma (UK)
Orphan Indication	Treatment of familial adenomatous polyposis
COMP Opinion date	08/07/2009
Orphan Designation date	08/10/2009

Active substance	Expanded human allogeneic mesenchymal adult stem cells extracted from adipose tissue (eASCs, adipose derived stem cells)
Sponsor	Cellerix S.A.
Orphan Indication	Treatment of anal fistula
COMP Opinion date	08/07/2009
Orphan Designation date	08/10/2009

Active substance	Low molecular weight dextran sulfate
Sponsor	TikoMed AB
Orphan Indication	Prevention of graft rejection during pancreatic islet transplantation
COMP Opinion date	08/07/2009
Orphan Designation date	09/10/2009

Active substance	Masitinib mesilate
Sponsor	AB Science
Orphan Indication	Treatment of pancreatic cancer
COMP Opinion date	02/09/2009
Orphan Designation date	28/10/2009

Active substance	Pasireotide
Sponsor	Novartis Europharm Limited
Orphan Indication	Treatment of Cushing's disease
COMP Opinion date	08/07/2009
Orphan Designation date	08/10/2009

Active substance	Pasireotide
Sponsor	Novartis Europharm Limited
Orphan Indication	Treatment of acromegaly
COMP Opinion date	08/07/2009
Orphan Designation date	08/10/2009

Active substance	Pomalidomide
Sponsor	Celgene Europe Limited
Orphan Indication	Treatment of multiple myeloma
COMP Opinion date	08/07/2009
Orphan Designation date	08/10/2009

Active substance	Recombinant antibody construct against human CD30 and CD16A
Sponsor	Affimed Therapeutics AG
Orphan Indication	Treatment of Hodgkin lymphoma
COMP Opinion date	08/07/2009
Orphan Designation date	09/10/2009

Active substance	Recombinant human serum amyloid P
Sponsor	RegPak Biopharma
Orphan Indication	Prevention of scarring post glaucoma filtration
COMP Opinion date	08/07/2009
Orphan Designation date	09/10/2009

Active substance	Sequence-modified recombinant human factor VIIa
Sponsor	Bayer Schering Pharma AG
Orphan Indication	Treatment of haemophilia A
COMP Opinion date	08/07/2009
Orphan Designation date	09/10/2009

Active substance	Sequence-modified recombinant human factor VIIa
Sponsor	Bayer Schering Pharma AG

Orphan Indication	Treatment of haemophilia B
COMP Opinion date	08/07/2009
Orphan Designation date	08/10/2009