22 July 2011
EMA/COMP/399953/2011 Corr.
Human Medicines Development and Evaluation

Monthly report

Committee for Orphan Medicinal Products (COMP)

6-8 July 2011

The Committee for Orphan Medicinal Products held its 125th plenary meeting on 6-8 July 2011.

During this meeting the Committee agreed to support the initiative to publish the prevalence figures and information on their sources for conditions that have been subject of orphan designations. The initiative aims at increasing transparency on orphan designation and acknowledging the work done for the prevalence calculation in the context of designation, which can be helpful as initial reference for future applications. This initiative will be developed progressively and will focus on those conditions for which the quality and volume of data allows for a stable estimate. The information will be published on the Agency website and updated regularly. The Committee discussed other initiatives in this field that will be further developed.

Orphan medicinal product designation

The COMP adopted 16 positive opinions recommending the following medicines for designation as orphan medicinal products to the European Commission:

For the following medicines the review began on 11 April 2011 with an active review time of 89 days:

- **Eflornithine** for treatment of neuroblastoma; Cancer Prevention Pharma Ltd.
- **Genetically modified *Lactococcus lactis* bacteria containing the human *trefoil factor 1* gene** for prevention of oral mucositis in head and neck cancer patients undergoing radiation therapy; ActoGeniX N.V.
- **Heterologous human adult liver-derived stem cells** for treatment of ornithine transcarbamylase deficiency, Fresenius Medical Care Deutschland GmbH.
- **Macitentan** for treatment of pulmonary arterial hypertension; Actelion Registration Limited.
- **NH_2-Cys-Ser-Ser-Val-Thr-Ala-Trp-Thr-Thr-Gly-Cys-Gly-$CONH_2$** for treatment of traumatic spinal cord injury; PHARMAXON.

7 Westferry Circus ● Canary Wharf ● London E14 4HB ● United Kingdom
Telephone +44 (0)20 7418 8400 **Facsimile** +44 (0)20 7523 7040
E-mail info@ema.europa.eu **Website** www.ema.europa.eu
An agency of the European Union

For the following medicines the review began on 10 June 2011:

- **2,2'-{2-[(1R)-1-({[(2,5-dichlorobenzoyl)amino]acetyl}amino)-3-methylbutyl]-5-oxo-1,3,2-dioxaborolane-4,4-diyl}diacetic acid** for treatment of multiple myeloma, Takeda Global Research and Development Centre (Europe) Ltd (with an active review time of 29 days).
- **20-pentaerythritol poly (oxy-1,2-ethanediyl)-carboxymethyl-glycinate-7-ethyl-10-hydroxycamptothecine 10-[1,4'-bipiperidine]-1'-carboxylate** for treatment of ovarian cancer, Nektar Therapeutics UK Ltd (with an active review time of 29 days).
- **Dinaciclib** for treatment of chronic lymphocytic leukaemia Merck Sharp & Dohme Limited (adopted via written procedure with an active review time of 36 days).
- **Kifunensine** for treatment of alpha-sarcoglycanopathy; Généthon (adopted via written procedure with an active review time of 36 days).
- **Kifunensine** for treatment of beta-sarcoglycanopathy; Généthon (adopted via written procedure with an active review time of 36 days).
- **Kifunensine** for treatment of delta-sarcoglycanopathy; Généthon (adopted via written procedure with an active review time of 36 days).
- **Kifunensine** for treatment of gamma-sarcoglycanopathy; Généthon (adopted via written procedure with an active review time of 36 days).
- **Recombinant human galactocerebrosidase** for treatment of globoid cell leukodystrophy (Krabbe disease); ACE Biosciences A/S (with an active review time of 29 days).
- **Reparixin** for prevention of graft rejection in pancreatic islet transplantation; Dompé S.p.A. (with an active review time of 29 days).
- **Resminostat** for treatment of hepatocellular carcinoma; 4SC AG (adopted via written procedure with an active review time of 36 days).
- **Smilagenin** for treatment of amyotrophic lateral sclerosis; Phytopharm plc (adopted via written procedure with an active review time of 36 days).

Public summaries of opinions will be available on the Agency's website following adoption of the respective decisions on orphan designation by the European Commission.

Other information on the orphan medicinal product designation

The COMP adopted 11 lists of questions on initial applications. These applications will be discussed again at the next COMP meeting prior to adoption of the opinion.

Oral hearings

5 oral hearings took place.

Withdrawals of applications for orphan medicinal product designation

The COMP noted that 2 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 meeting is provided in Annex 2.

Applications for marketing authorisation for orphan medicinal products

Details of those designated orphan medicinal products that have been subject of a new European Union marketing authorisation application through the centralised procedure since the last COMP plenary meeting are provided in Annex 3.

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 Agency's 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 1 opinion recommending to the European Commission that the following orphan medicinal product be kept in the EU registry of orphan medicinal products:

- **Everolimus (Votubia)** for treatment of tuberous sclerosis; Novartis Europharm Limited.

Upcoming meetings

- The 126th meeting of the COMP will be held on 6-8 September 2011.

Other matters

The main topics addressed during the meeting related to:

- 1 Protocol Assistance letter was adopted.

Note

This monthly report, together with other information on the work of the European Medicines Agency, can be found on the Agency's website: www.ema.europa.eu

Contact our press officer

Monika Benstetter

Tel. +44 (0)20 7418 8427

E-mail: press@ema.europa.eu

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/sectors/pharmaceuticals/documents/community-register/html/index_en.htm

Annex 1

Overview for orphan medicinal product designation procedure since 2000

Year	Applications submitted	Applications discussed in reporting year	Positive COMP opinions	Applications withdrawn	Final negative COMP opinions	Designations granted by Commission
2011	79	88	66 (75%)	21 (24%)	1 (1%)	50
2010	174	176	123 (70%)	51 (29%)	2[2] (1%)	128
2009	164	137	113 (82%)	23 (17%)	1 (1%)	106
2008	119	118	86 (73%)	31 (26%)	1 (1%)	73
2007	125	117	97 (83%)	19 (16%)	1 (1%)	98
2006	104	103	81 (79%)	20 (19%)	2 (2%)	80
2005	118	118	88 (75%)	30 (25%)	0 (0%)	88
2004	108	101	75 (74%)	22 (22%)	4 (4%)	72
2003	87	96	54 (56%)	41 (43%)	1 (1%)	55
2002	80	76	43 (57%)	30 (39%)	3 (4%)	49
2001	83	92	64 (70%)	27 (29%)	1 (1%)	64
2000	72	32	26 (81%)	6 (19%)	0 (0%)	14
Total	**1313**	**1254**	**916 (73%)**	**321 (26%)**	**17 (1%)**	**877**

[2] One more opinion was re-adopted in 2010 following the appeal to a negative opinion from 2009

Annex 2

Medicinal products granted a European Union designation as orphan medicinal product by the European Commission since the June 2011 COMP monthly report

Active substance	**Adeno-associated viral vector serotype 9 containing the human sulfamidase gene**
Sponsor	Laboratorios del Dr. Esteve, S.A.
Orphan indication	Treatment of mucopolysaccharidosis type IIIA (Sanfilippo A syndrome)
COMP opinion date	7 April 2011
Orphan designation date	21 June 2011

Active substance	**Allogeneic T cells encoding an exogenous thymidine kinase gene**
Sponsor	LTKFarma
Orphan indication	Treatment of acute lymphoblastic leukaemia
COMP opinion date	7 April 2011
Orphan designation date	21 June 2011

Active substance	**Chimeric monoclonal antibody against GD2**
Sponsor	United Therapeutics Europe Ltd
Orphan indication	Treatment of neuroblastoma
COMP opinion date	7 April 2011
Orphan designation date	21 June 2011

Active substance	**Genetically modified human adenovirus encoding human PH20 hyaluronidase**
Sponsor	VCN Biosciences S.L.
Orphan indication	Treatment of pancreatic cancer
COMP opinion date	7 April 2011
Orphan designation date	21 June 2011

Active substance	**Human dermal fibroblasts cultured on a bioresorbable polyglactin mesh**
Sponsor	TMC Pharma Services Ltd
Orphan indication	Treatment of epidermolysis bullosa
COMP opinion date	9 March 2011
Orphan designation date	21 June 2011

Active substance	Human embryonic stem-cell-derived retinal pigment epithelial cells
Sponsor	TMC Pharma Services Ltd
Orphan indication	Treatment of Stargardt's disease
COMP opinion date	9 March 2011
Orphan designation date	21 June 2011

Active substance	Metronidazole
Sponsor	FORMAC Pharmaceuticals N.V.
Orphan Indication	Treatment of pouchitis
COMP opinion date	9 March 2011
Orphan Designation date	21 June 2011

Active substance	S-farnesylthiosalicylic acid
Sponsor	TMC Pharma Services Ltd
Orphan indication	Treatment of pancreatic cancer
COMP opinion date	9 March 2011
Orphan designation date	21 June 2011

Active substance	Sulfonated monophosphorylated mannose oligosaccharide
Sponsor	S-Cubed Limited
Orphan indication	Treatment of hepatocellular carcinoma
COMP opinion date	9 March 2011
Orphan designation date	21 June 2011

Active substance	Viral vector containing DNA encoding the human SMN protein
Sponsor	University of Sheffield
Orphan indication	Treatment of 5q spinal muscular atrophy
COMP opinion date	9 March 2011
Orphan designation date	21 June 2011

Annex 3

Designated orphan medicinal products that have been subject of a new European Union marketing authorisation application under the centralised procedure since the June 2011 COMP monthly report

Active substance	Invented name	Sponsor/applicant	EU designation number	Designated orphan indication
(R)-3-(4-(7H-pyrrolo[2,3-d]pyrimidin-4-yl)-1H-pyrazol-1-yl)-3-cyclopentylpropanenitrile phosphate	Jakavi[3]	Novartis Europharm Limited	EU/3/09/620	Treatment of myelofibrosis secondary to polycythaemia vera or essential thrombocythaemia
			EU/3/08/572	Treatment of chronic idiopathic myelofibrosis
Idebenone	SAN Idebenone	Santhera Pharmaceuticals (Deutschland) GmbH	EU/3/07/434	Treatment of Leber's hereditary optic neuropathy
Monoclonal antibody against human CD30 covalently linked to the cytotoxin monomethylauristatin E	Adcetris	Takeda Global Research and Development Centre (Europe) Ltd	EU/3/08/596	Treatment of Hodgkin lymphoma

[3] Corrigendum shows updated record for Jakavi