10 March 2011
EMA/COMP/134956/2011
Human Medicines Development and Evaluation

Monthly report

Committee for Orphan Medicinal Products (COMP)

8-9 March 2011

The Committee for Orphan Medicinal Products held its 121st plenary meeting on 8-9 March 2011.

Orphan medicinal product designation

The COMP adopted 6 positive opinions recommending the following medicines for designation as orphan medicinal products to the European Commission.

For the following medicines the review began on 10 January 2011 with an active review time of 59 days:

- **Human dermal fibroblasts cultured on a bioresorbable polyglactin mesh** for treatment of epidermolysis bullosa; TMC Pharma Services Ltd.
- **Human embryonic stem-cell-derived retinal pigment epithelial cells** for treatment of Stargardt's disease; TMC Pharma Services Ltd.
- **Metronidazole** for treatment of pouchitis; FORMAC Pharmaceuticals N.V.
- **S-farnesylthiosalicylic acid** for treatment of pancreatic cancer; TMC Pharma Services Ltd.
- **Sulfonated monophosphorylated mannose oligosaccharide** for treatment of hepatocellular carcinoma; S-Cubed Limited.
- **Viral vector containing DNA encoding the human SMN protein** for treatment of 5q spinal muscular atrophy; the University of Sheffield.

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.

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Other information on the orphan medicinal product designation

Lists of questions

The COMP adopted 1 list of questions on initial application. The application will be discussed again at the next COMP meeting prior to adoption of the opinion.

Oral hearings

4 oral hearings took place.

Withdrawals of applications for orphan medicinal product designation

The COMP noted that 4 applications for orphan medicinal product designation were withdrawn.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.

Upcoming meetings

- The 122nd meeting of the COMP will be held on 6-7 April 2011.

Other matters

The main topics addressed during the meeting related to:

- 2 Protocol Assistance letters were 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

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

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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	20	38	29 (76%)	9 (24%)	0 (0%)	18
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	**1254**	**1204**	**879 (73%)**	**309 (26%)**	**16 (1%)**	**845**

[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 February 2011 COMP monthly report

Active substance	**Allogeneic aortic endothelial cells cultured in a porcine gelatin matrix**
Sponsor	Gregory Fryer Associates Ltd
Orphan indication	Prevention of arteriovenous access failure in haemodialysis patients
COMP opinion date	8 December 2010
Orphan designation date	23 February 2011

Active substance	**Autologous tumour-derived immunoglobulin idiotype coupled to keyhole limpet hemocyanin**
Sponsor	Analytica International Inc.
Orphan indication	Treatment of mantle cell lymphoma
COMP opinion date	10 November 2010
Orphan designation date	23 February 2011

Active substance	**Axitinib**
Sponsor	Pfizer Limited
Orphan indication	Treatment of renal cell carcinoma
COMP opinion date	8 December 2010
Orphan designation date	23 February 2011

Active substance	**Deferiprone**
Sponsor	Apotex Europe B.V.
Orphan indication	Treatment of sickle cell disease
COMP opinion date	10 November 2010
Orphan designation date	23 February 2011

Active substance	**Dry extract from birch bark (DER 0.1-0.2:1), extraction solvent n-heptane 95% (V/V)**
Sponsor	Birken GmbH
Orphan indication	Treatment of epidermolysis bullosa
COMP opinion date	8 December 2010
Orphan designation date	23 February 2011

Active substance	**Doxorubicin hydrochloride (in heat-sensitive liposomes)**
Sponsor	Biological Consulting Europe Ltd.
Orphan Indication	Treatment of hepatocellular carcinoma
COMP opinion date	10 November 2010
Orphan Designation date	23 February 2011

Active substance	Human plasmin
Sponsor	Talecris Biotherapeutics GmbH
Orphan indication	Treatment of acute peripheral arterial occlusion
COMP opinion date	10 November 2010
Orphan designation date	23 February 2011

Active substance	Maytansinoid-conjugated humanised monoclonal antibody against CD56
Sponsor	ImmunoGen Europe Limited
Orphan indication	Treatment of multiple myeloma
COMP opinion date	10 November 2010
Orphan designation date	

Active substance	Nimorazole
Sponsor	Azanta A/S
Orphan indication	Treatment of squamous cell carcinoma of the head and neck in patients undergoing radiotherapy
COMP opinion date	10 November 2010
Orphan designation date	23 February 2011

Active substance	Paclitaxel (aqueous gel)
Sponsor	BTG plc
Orphan indication	Treatment of oesophagus carcinoma
COMP opinion date	8 December 2010
Orphan designation date	23 February 2011

Active substance	Paquinimod
Sponsor	Active Biotech AB
Orphan indication	Treatment of systemic sclerosis
COMP opinion date	10 November 2010
Orphan designation date	23 February 2011

Active substance	Pegylated B-domain-deleted sequence-modified recombinant human factor VIII
Sponsor	Bayer Schering Pharma AG
Orphan indication	Treatment of haemophilia A
COMP opinion date	8 December 2010
Orphan designation date	23 February 2011

Active substance	Plitidepsin
Sponsor	Pharma Mar SA Sociedad Unipersonal
Orphan indication	Treatment of post-essential thrombocythaemia myelofibrosis
COMP opinion date	10 November 2010
Orphan designation date	23 February 2011

Active substance	Plitidepsin
Sponsor	Pharma Mar SA Sociedad Unipersonal
Orphan indication	Treatment of post-polycythaemia vera myelofibrosis
COMP opinion date	10 November 2010
Orphan designation date	23 February 2011

Active substance	Plitidepsin
Sponsor	Pharma Mar SA Sociedad Unipersonal
Orphan indication	Treatment of primary myelofibrosis
COMP opinion date	10 November 2010
Orphan designation date	23 February 2011

Active substance	Recombinant human histone H1.3 and recombinant human N-bis-met-histone H1.3
Sponsor	SymbioTec GmbH
Orphan indication	Treatment of acute lymphoblastic leukaemia
COMP opinion date	10 November 2010
Orphan designation date	23 February 2011

Active substance	Sodium thiosulphate
Sponsor	Promedipharm GmbH
Orphan indication	Treatment of calciphylaxis
COMP opinion date	8 December 2010
Orphan designation date	23 February 2011

Active substance	Tasimelteon
Sponsor	Vanda Pharmaceuticals Limited
Orphan indication	Treatment of non-24-hour sleep-wake disorders in blind people with no light perception
COMP opinion date	10 November 2010
Orphan designation date	23 February 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 February 2011 COMP monthly report

Active substance	Invented name	Sponsor/applicant	EU designation number	Designated orphan indication
Romidepsin (INN) (E)-(1S,4S,10S,21R)-7-[(Z)-ethylidene]-4,21-diisopropyl-2-oxa-12,13-dithia-5,8,20,23-tetraazabicyclo[8.7.6]tricos-16-ene-3,6,9,19,22-pentone	TBC	Celgene Europe Limited;	EU/3/05/328	Treatment of peripheral T-cell lymphoma (nodal, other extranodal and leukaemic/disseminated)