12 November 2010
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Human Medicines Development and Evaluation

Monthly report

Committee for Orphan Medicinal Products (COMP)

9-10 November 2010

The Committee for Orphan Medicinal Products held its 117th plenary meeting on 9-10 November 2010.

Orphan medicinal product designation

The COMP adopted 12 positive opinions recommending the following medicines for designation as orphan medicinal products to the European Commission:

For the following medicines the review began on 13 August 2010 with an active review time of 90 days:

- **Deferiprone** for treatment of sickle cell disease, Apotex Europe B.V.
- **Nimorazole** for treatment of squamous cell carcinoma of the head and neck in patients undergoing radiotherapy, Azanta A/S.
- **Paquinimod** for treatment of systemic sclerosis, Active Biotech Research AB.
- **Recombinant human histone H1.3 and recombinant human N-bis-met-histone H1.3** for treatment of acute lymphoblastic leukaemia, SymbioTec GmbH.
- **Tasimelteon** for treatment of non-24-hour sleep-wake disorder in blind people with no light perception, Vanda Pharmaceuticals Limited.

For the following medicines the review began on 10 September 2010 with an active review time of 62 days:

- **Autologous tumour-derived immunoglobulin idiotype coupled to keyhole limpet hemocyanin** for treatment of mantle cell lymphoma, Analytica International Inc.
- **Doxorubicin hydrochloride (in heat-sensitive liposomes)** for treatment of hepatocellular carcinoma, Biological Consulting Europe Ltd.
- **Human plasmin** for treatment of acute peripheral arterial occlusion, Talecris Biotherapeutics GmbH.

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- **Maytansinoid-conjugated humanized monoclonal antibody against CD56** for treatment of multiple myeloma, ImmunoGen Europe Limited.
- **Plitidepsin** for treatment of primary myelofibrosis, Pharma Mar SA Sociedad Unipersonal.
- **Plitidepsin** for treatment of post-polycythaemia vera myelofibrosis, Pharma Mar SA Sociedad Unipersonal.
- **Plitidepsin** for treatment of post-essential thrombocythaemia myelofibrosis, Pharma Mar SA Sociedad Unipersonal.

Other information on the orphan medicinal product designation

Lists of questions

The COMP adopted 4 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

7 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.

Upcoming meetings

- The 118th meeting of the COMP will be held on 7-8 December 2010.

Other matters

The main topics addressed during the meeting related to:

- COMP working procedures. The Committee is starting a process to improve its efficiency and to address better working procedures adapted to the increased number of applications and their growing complexity.
- Adoption of the report from the Informal COMP meeting held on 30 September – 1 October 2010 in Antwerp. The main topics discussed were the analysis of annual reports and the impact of new advances in biology in the definition of orphan conditions.

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

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

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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
2010	149	144	117 (81%)	46 (32%)	3 (2%)	98
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	**1209**	**1134**	**844 (74%)**	**295 (26%)**	**17 (1%)**	**797**

Annex 2

Medicinal products granted a European Union designation as orphan medicinal product by the European Commission since the October 2010 COMP monthly report

Active substance	16-base single-stranded peptide nucleic acid oligonucleotide linked to a 7-amino acid peptide
Sponsor	Biogenera srl
Orphan indication	Treatment of medulloblastoma
COMP opinion date	8 July 2010
Orphan designation date	1 October 2010

Active substance	(3S)-3-{4-[7-(aminocarbonyl)-2H-indazol-2-yl] phenyl} piperidine (tosylate monohydrate salt)
Sponsor	Merck Sharp & Dohme Limited
Orphan indication	Treatment of mantle cell lymphoma
COMP opinion date	16 July 2010
Orphan designation date	1 October 2010

Active substance	(*S*)-10-[(dimethylamino)methyl]-4-ethyl-9-hydroxy-4-*O*-[alpha-(2", 4", 5", 7"-tetranitro-9"-fluorenylideneaminooxy)propionyl]-1*H*-pyrano[3', 4', 6', 7']indolizino[1,2-beta]-quinoline-3, 14-(4*H*, 12*H*)-dione, hydrochloride
Sponsor	TLC Biopharmaceuticals B.V.
Orphan Indication	Treatment of hepatocellular carcinoma
COMP opinion date	8 July 2010
Orphan Designation date	1 October 2010

Active substance	Ambrisentan
Sponsor	Gilead Sciences International Ltd
Orphan indication	Treatment of idiopathic pulmonary fibrosis
COMP opinion date	8 July 2010
Orphan designation date	1 October 2010

Active substance	Ciclosporin
Sponsor	NeuroVive Pharmaceuticals AB
Orphan indication	Treatment of moderate or severe closed traumatic brain injury
COMP opinion date	8 July 2010
Orphan designation date	1 October 2010

Active substance	Maytansinoid-conjugated humanised monoclonal antibody against CD56
Sponsor	ImmunoGen Europe Limited
Orphan indication	Treatment of small cell lung cancer
COMP opinion date	8 July 2010
Orphan designation date	1 October 2010

Active substance	N-(6-(2-aminophenylamino)-6-oxohexyl)-4-methylbenzamide
Sponsor	Repligen Europe Limited
Orphan indication	Treatment of Friedreich's ataxia
COMP opinion date	8 July 2010
Orphan designation date	1 October 2010

Active substance	N-tert-butyl-3-[(5-methyl-2-{[4-(2-pyrrolidin-1-ylethoxy)phenyl]amino}pyrimidin-4-yl)amino] benzenesulfonamide dihydrochloride monohydrate
Sponsor	Dr Ulrich Granzer
Orphan indication	Treatment of primary myelofibrosis
COMP opinion date	16 July 2010
Orphan designation date	1 October 2010

Active substance	Pralatrexate
Sponsor	Allos Therapeutics Limited
Orphan indication	Treatment of Hodgkin's lymphoma
COMP opinion date	16 July 2010
Orphan designation date	1 October 2010

Active substance	Recombinant humanised monoclonal antibody to human Nogo-A protein of the IgG1/kappa class
Sponsor	Glaxo Group Limited
Orphan indication	Treatment of amyotrophic lateral sclerosis
COMP opinion date	8 July 2010
Orphan designation date	1 October 2010

Active substance	Recombinant humanised anti-human interleukin-1 beta monoclonal antibody
Sponsor	XOMA Ireland Ltd
Orphan indication	Treatment of Behçet's disease
COMP opinion date	8 July 2010
Orphan designation date	1 October 2010

Active substance	Synthetic double-stranded short interfering RNA oligonucleotide directed against pro-opiomelanocortin
Sponsor	UKR Regulatory Affairs Ltd
Orphan indication	Treatment of adrenocorticotropin-dependent Cushing's syndrome
COMP opinion date	8 July 2010
Orphan designation date	1 October 2010