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Committee for Orphan Medicinal Products (COMP)

Committee for Orphan Medicinal Products (COMP) meeting report on the review of applications for orphan designation

December 2014

The Committee for Orphan Medicinal Products held its 162nd plenary meeting on 9-11 December 2014.

Orphan medicinal product designation

Positive opinions

The COMP adopted 22 positive opinions recommending the following medicines for designation as orphan medicinal products to the European Commission (EC):

1. Opinions adopted at the second COMP discussion, following the sponsor's response to the COMP list of questions:

- A lentiviral vector pseudotyped by the Indiana serotype of the vesicular stomatitis virus G protein encoding an antigen derived from the Tax, HBZ, p12I and p30II HTLV-1 proteins for treatment of adult T-cell leukaemia/lymphoma, THERAVECTYS
- A lentiviral vector pseudotyped by the New-Jersey serotype of the vesicular stomatitis virus G protein encoding an antigen derived from the Tax, HBZ, p12I and p30II HTLV-1 proteins for treatment of adult T-cell leukaemia/lymphoma, THERAVECTYS
- Adenoviral vector serotype 5 containing the vascular endothelial growth factor D isoform (preprocessed short form) from a CMV promoter for treatment of placental insufficiency, Magnus Invention Management Ltd
- Allogeneic, umbilical cord blood-derived, ex vivo-expanded, haematopoietic CD133+ cells / allogeneic, umbilical cord blood-derived, non-expanded, haematopoietic CD133- cells for treatment of acute myeloid leukaemia, Regulatory Resources Group Ltd
- Chimeric monoclonal antibody to O-acetyl-GD2 antigen for treatment of neuroblastoma, Atlab Pharma SAS
- Emtricitabine for treatment of Aicardi-Goutières syndrome, Dr Yanick Crow

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- Herpes simplex type 1 virus containing cellular *B-myb* gene as tumour-specific promoter for treatment of pancreatic cancer, Karcinolys S.A.S
- Pentosan polysulfate sodium for treatment of interstitial cystitis, Dr Ulrich Granzer
- Recombinant human aspartylglucosaminidase for treatment of aspartylglucosaminuria, ACE Biosciences A/S
- Sodium thiosulfate for treatment for calciphylaxis, Hope Pharmaceuticals, Ltd
- Tenofovir disoproxil fumarate for treatment of Aicardi-Goutières syndrome, Dr Yanick Crow

2. Opinions adopted at the first COMP discussion:

- Adeno-associated viral vector serotype 8 containing the human factor-VII gene for treatment of congenital factor VII deficiency, Professor Edward G. Tuddenham
- Allogeneic peripheral blood mononuclear cells induced to an early apoptotic state for prevention of graft-versus-host disease, Richardson Associates Regulatory Affairs Ltd
- Ceftriaxone for treatment of spinocerebellar ataxia, Ospedale San Raffaele s.r.l.
- Chimeric fusion protein of recombinant human alpha-N-acetylglucosaminidase and human insulin-like growth factor 2 for treatment of mucopolysaccharidosis type IIIB (Sanfilippo B syndrome), BioMarin Europe Ltd.
- Humanised Fc engineered monoclonal antibody against CD19 for treatment of diffuse large B-cell lymphoma, MorphoSys AG
- N-methyl-4-({4-[({3-methyl(methylsulfonyl)aminopyrazin-2-yl}methyl)amino]-5-(trifluoromethyl)pyrimidin-2-yl}amino)benzamide hydrochloride for treatment of ovarian cancer, TMC Pharma Services Ltd
- Pegylated recombinant arginine deiminase for treatment of malignant mesothelioma, Designerx Europe Limited
- Ponatinib hydrochloride for treatment of gastrointestinal stromal tumours, ARIAD Pharma Ltd
- Recombinant human alkaline phosphatase for treatment of hypophosphatasia, AM-Pharma BV
- Sodium valproate for treatment of Wolfram syndrome, Alan Boyd Consultants Ltd
- Synthetic signal peptide of human Mucin-1 (amino acids 1-21) for treatment of plasma cell myeloma, Richardson Associates Regulatory Affairs Ltd

Public summaries of opinions will be available on the EMA website following adoption of the respective decisions on orphan designation[1] by the European Commission.

Lists of questions

The COMP adopted 21 lists of questions on initial applications. These applications will be discussed again at the next COMP meeting prior to the adoption of an opinion.

[1] Details of all orphan designations granted to date by the European Commission are entered in the EU Register of Orphan Medicinal Products

Oral hearings

15 oral hearings took place.

Withdrawals of applications for orphan medicinal product designation

The COMP noted that 11 applications for orphan medicinal product designation were withdrawn.

Detailed information on the orphan designation procedures

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 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 (EU) marketing authorisation application through the centralised procedure since the last COMP plenary meeting are provided in Annex 3.

Details on the authorised orphan medicinal products can be found on the EMA website.

Article 5(12) (b) 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 product be kept in the EU registry of orphan medicinal product:

- OFEV (nintedanib) for treatment of idiopathic pulmonary fibrosis; Boehringer Ingelheim International GmbH (EU/3/13/1123)
- Cerdelga ((1R,2R)-octanoic acid[2-(2',3'-dihydro-benzo[1,4] dioxin-6'-yl)-2-hydroxy-1-pyrrolidin-1-ylmethyl-ethyl]-amide-L-tartaric acid salt) for treatment of Gaucher disease; Genzyme Europe BV (EU/3/07/514)

Other matters

The main topics addressed during the meeting related to:

- Protocol assistance advice

Upcoming meetings

- The 163rd meeting of the COMP will be held on 7-9 January 2015

Note

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

Contact our press officer

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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	EC designations	Orphan medicinal products[2] authorised	Orphan designations included in authorised therapeutic indication
2014	327	259	196 (76%)	61 (24%)	2 (1%)	160	12	13
2013	201	197	136 (69%)	60 (30%)	1 (1%)	136	7	8
2012	197	192	139 (72%)	52 (27%)	1 (1%)	148	10	12
2011	166	158	111 (70%)	45 (29%)	2 (1%)	107	5	5
2010	174	176	123 (70%)	51 (29%)	2 (1%)	128	4	4
2009	164	136	113 (83%)	23 (17%)	0 (0%)	106	9	9
2008	119	118	86 (73%)	31 (26%)	1 (1%)	73	6	7
2007	125	117	97 (83%)	19 (16%)	1 (1%)	98	13	13
2006	104	103	81 (79%)	20 (19%)	2 (2%)	80	9	11
2005	118	118	88 (75%)	30 (25%)	0 (0%)	88	4	4
2004	108	101	75 (74%)	22 (22%)	4 (4%)	73	6	6
2003	87	96	54 (56%)	41 (43%)	1 (1%)	55	5	5
2002	80	75	43 (57%)	30 (40%)	2 (3%)	49	4	4
2001	83	90	62 (70%)	27 (29%)	1 (1%)	64	3	3
2000	72	32	26 (81%)	6 (19%)	0 (0%)	14	0	0
Total	**2125**	**1968**	**1430 (73%)**	**518 (26%)**	**20 (1%)**	**1379**	**97**	**104**

[2] Number of authorised orphan medicinal products may cover more than one orphan designation

Annex 2

Medicinal products granted a European Union designation as orphan medicinal product by the European Commission since the November 2014 COMP monthly report

Active substance	Orphan indication	Sponsor	COMP opinion date	EC designation date
(2R,3S)-2-(4-cyclopentylaminophenyl)-1-(2-fluoro-6-methylbenzoyl)piperidine-3-carboxylic acid(4-methyl-3-trifluoromethylphenyl)amide	Treatment of granulomatosis with polyangiitis	ChemoCentryx Limited	9 October 2014	19 November 2014
(2R,3S)-2-(4-cyclopentylaminophenyl)-1-(2-fluoro-6-methylbenzoyl)piperidine-3-carboxylic acid(4-methyl-3-trifluoromethylphenyl)amide	Treatment of microscopic polyangiitis	ChemoCentryx Limited	9 October 2014	19 November 2014
(3S)-1-azabicyclo[2.2.2]oct-3-yl{2-[2-(4-fluorophenyl)-1,3-thiazol-4-yl]propan-2-yl}carbamate	Treatment of Gaucher disease	Genzyme Europe BV	9 October 2014	19 November 2014
1-(6-benzothiazolylsulfonyl)-5-chloro-1H-indole-2-butanoic acid	Treatment of systemic sclerosis	Inventiva	9 October 2014	19 November 2014
1-(6-benzothiazolylsulfonyl)-5-chloro-1H-indole-2-butanoic acid	Treatment of idiopathic pulmonary fibrosis	Inventiva	9 October 2014	19 November 2014
4-[[(1S,4S)-5-[[4-[4-(oxazol-2-yl)phenoxy]phenyl]methyl]-2,5-diazabicyclo[2.2.1]hept-2-yl]methyl]benzoic acid	Treatment of cystic fibrosis	Coté Orphan Consulting UK Limited	9 October 2014	19 November 2014
A combination of H-Lys-Lys-Gly-Pro-Arg-Cys(SH)-Leu-Thr-Arg-Tyr-Tyr-Ser-Ser-Phe-Val-Asn-Met-Glu-Gly-Lys-Lys-OH and H-Lys-Lys-Gly-Asp-Asn-Ile-Met-Val-Thr-Phe-Arg-Asn-Gln-Ala-Ser-Arg-Pro-Tyr-Gly-Lys-Lys-OH	Treatment of haemophilia A	Apitope International NV	9 October 2014	19 November 2014

Active substance	Orphan indication	Sponsor	COMP opinion date	EC designation date
Adeno-associated viral vector serotype 8 containing the human *MD1* gene	Treatment of Duchenne muscular dystrophy	Généthon	9 October 2014	19 November 2014
Arimoclomol citrate	Treatment of Niemann-Pick disease, type C	Orphazyme ApS	9 October 2014	19 November 2014
Ataluren	Treatment of mucopolysaccharidosis type I	PTC Therapeutics, Limited	9 October 2014	19 November 2014
Bazedoxifene acetate	Treatment of hereditary haemorrhagic telangiectasia	Consejo Superior de Investigaciones Cientificas (CSIC)	9 October 2014	19 November 2014
Chloroquine	Treatment of glioma	DualTpharma B.V.	9 October 2014	19 November 2014
Dantrolene sodium	Treatment of malignant hyperthermia	Eagle Laboratories Ltd	9 October 2014	19 November 2014
Diaspirin cross-linked haemoglobin	Treatment of hepatocellular carcinoma	New B Innovation (UK) Limited	9 October 2014	19 November 2014
Donor T lymphocytes depleted ex vivo of host alloreactive T cells using photodynamic treatment	Treatment of acute myeloid leukaemia	Kiadis Pharma Netherlands B.V	9 October 2014	19 November 2014
Humanised IgG1 monoclonal antibody against human eotaxin-2	Treatment of systemic sclerosis	CBR Biotech Strategies GmbH	9 October 2014	19 November 2014
Imatinib	Treatment of acute respiratory distress syndrome	Numedicus Limited	9 October 2014	19 November 2014
Mexiletine hydrochloride	Treatment of myotonic disorders	Temmler Pharma GmbH & Co. KG	9 October 2014	19 November 2014
Olaptesed pegol	Treatment of glioma	Noxxon Pharma AG	9 October 2014	19 November 2014
Palovarotene	Treatmentof fibrodysplasia ossificans progressiva	Medpace Germany GmbH	9 October 2014	19 November 2014

Active substance	Orphan indication	Sponsor	COMP opinion date	EC designation date
Pentosan polysulfate sodium	Treatment of mucopolysaccharidosis type I	Plexcera Therapeutics EU Limited	9 October 2014	19 November 2014
Pro-Pro-Thr-Val-Pro-Thr-Arg	Treatment of xeroderma pigmentosum	Prof Alain Taieb	9 October 2014	19 November 2014
Recombinant human pentraxin-2	Treatment of post-essential thrombocythaemia myelofibrosis	FGK Representative Service GmbH	9 October 2014	19 November 2014
Recombinant human pentraxin-2	Treatment of primary myelofibrosis	FGK Representative Service GmbH	9 October 2014	19 November 2014
Recombinant human pentraxin-2	Treatment of post-polycythaemia vera myelofibrosis	FGK Representative Service GmbH	9 October 2014	19 November 2014
Selinexor	Treatment of plasma cell myeloma	Clinipace GmbH	9 October 2014	19 November 2014
Selinexor	Treatment of chronic lymphocytic leukaemia / small lymphocytic lymphoma	Clinipace GmbH	9 October 2014	19 November 2014
Siponimod	Treatment of dermatomyositis	Novartis Europharm Limited	9 October 2014	19 November 2014
Siponimod	Treatment of polymyositis	Novartis Europharm Limited	9 October 2014	19 November 2014

Annex 3

Designated orphan medicinal products that have been subject of a new European Union marketing authorisation application under the centralised procedure since the November 2014 COMP monthly report

Active substance	Designated orphan indication	Sponsor/applicant	EU designation number
Isavuconazonium sulfate	Treatment of invasive aspergillosis	Basilea Medical Ltd	EU/3/14/1284
Isavuconazonium sulfate	Treatment of mucormycosis	Basilea Medical Ltd	EU/3/14/1276
Recombinant human parathyroid hormone	Treatment of hypoparathyroidism	NPS Pharma UK Ltd	EU/3/13/1210