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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

January 2015

The Committee for Orphan Medicinal Products held its 163rd plenary meeting on 7-9 January 2015.

Orphan medicinal product designation

Positive opinions

The COMP adopted 19 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:

- 3-[2-(4-carbamimidoyl-phenylcarbamoyl)-5-methoxy-4-vinyl-phenyl]-6-(cyclopropylmethyl-carbamoyl)-pyridine-2-carboxylic acid for treatment of hereditary angioedema, BioCryst UK Ltd.
- Chimeric group B adenovirus (11p/3) with deletions in the E3 and E4 regions for treatment of ovarian cancer, PsiOxus Therapeutics Ltd
- Fibrinogen-coated albumin spheres for treatment of Ebola virus disease, Fibreu Limited
- Nitroglycerin for treatment of systemic sclerosis, Covis Pharma S.à.r.l.
- Sevuparin sodium for treatment of sickle cell disease, Dilaforette AB

2. Opinions adopted at the first COMP discussion:

- 2'-*O*-methyl phosphorothioate RNA oligonucleotide, 5'-m^5CUGm^5CUGm^5CUGm^5CUGm^5CUGm^5CUGm^5CUG-3' for treatment of Huntington's disease, Prosensa Therapeutics B.V.
- 505 amino acid protein, corresponding to amino acids 2-506 of the wild type human histidyl-tRNA synthetase for treatment of facioscapulohumeral muscular dystrophy, Voisin Consulting S.A.R.L.
- 5-hydroxymethyl-2-furfural for treatment of sickle cell disease, Baxter Innovations GmbH

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- Allogeneic CD4+ and CD8+ T lymphocytes ex vivo incubated with synthetic peptides of the viral antigens of cytomegalovirus, adenovirus and Epstein-Barr virus for treatment of cytomegalovirus infection following haematopoietic stem cell transplantation, Miltenyi Biotec GmbH
- Allogeneic CD4+ and CD8+ T lymphocytes ex vivo incubated with synthetic peptides of the viral antigens of cytomegalovirus, adenovirus and Epstein-Barr virus for treatment of Epstein-Barr virus infection following haematopoietic stem cell transplantation, Miltenyi Biotec GmbH
- Allogeneic CD4+ and CD8+ T lymphocytes ex vivo incubated with synthetic peptides of the viral antigens of cytomegalovirus, adenovirus and Epstein-Barr virus for treatment of adenovirus infection following haematopoietic stem cell transplantation, Miltenyi Biotec GmbH
- Alvocidib for treatment of acute myeloid leukaemia, Theorem Clinical Research GmbH
- *Lactobacillus reuteri* for prevention of necrotising enterocolitis, Infant Bacterial Therapeutics
- Mazindol for treatment of narcolepsy, HAC Pharma
- Myriocin for treatment of retinitis pigmentosa, Nanovector s.r.l.
- N-(3-(4-(3-(diisobutylamino)propyl)piperazin-1-yl)propyl)-1H-benzo[d]imidazol-2-amine disulphate salt for treatment of progressive supranuclear palsy, AlzProtect sas
- Olaratumab for treatment of soft tissue sarcoma, Eli Lilly Nederland B.V.
- Recombinant human glutamate oxaloacetate transaminase 1 for treatment of glioma, Impasara Ltd
- Ulocuplumab for treatment of acute myeloid leukaemia, Bristol-Myers Squibb Pharma EEIG

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

Oral hearings

18 oral hearings took place.

Withdrawals of applications for orphan medicinal product designation

The COMP noted that 14 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.

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

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

- Holoclar (*Ex vivo* expanded autologous human corneal epithelium containing stem cells) for treatment of corneal lesions, with associated corneal (limbal) stem cell deficiency, due to ocular burns; Chiesi Farmaceutici S.p.A. (EU/3/08/579)

Other matters

The main topics addressed during the meeting related to:

- Protocol assistance advice

Upcoming meetings

- The 164th meeting of the COMP will be held on 10-12 February 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
2015	1	35	19 (54%)	16 (46%)	0 (0%)	0	0	0
2014	329	259	196 (76%)	61 (24%)	2 (1%)	187	15	16
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	**2128**	**2003**	**1449 (72%)**	**534 (27%)**	**20 (1%)**	**1406**	**100**	**107**

[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 December 2014 COMP monthly report

Active substance	Orphan indication	Sponsor	COMP opinion date	EC designation date
((E)-1-(4'-chlorophenyl)-3-(4-hydroxy-3-metoxyphenyl)prop-2-en-1-one)	Treatment of WHIM syndrome	Centre National de la Recherche Scientifique (CNRS)	13 November 2014	16 December 2014
(1S,4R,5R,7S)-3,4-dibenzyl-2-oxo-6,8-dioxa-3-azabyciclo[3.2.1]octane-7-carboxylic acid-L-lysine	Treatment of neurotrophic keratitis	MIMETECH S.r.l.	13 November 2014	16 December 2014
1-(2-isopropoxyethyl)-2-thioxo-1,2,3,5-tetrahydro-pyrrolo[3,2-d]pyrimidin-4-one	Treatment of multiple system atrophy	AstraZeneca AB	13 November 2014	16 December 2014
2-hydroxymethyl-2-methoxymethyl-1-azabicyclo[2,2,2]octan-3-one	Treatment of ovarian cancer	Aprea AB	13 November 2014	16 December 2014
5,5'-(4-(trifluromethyl)benzylazanediyl)bis(methylene) diquinolin-8-ol	Treatment of glioma	Prof. Olivier Blin	13 November 2014	16 December 2014
5-bromo-N-(prop-2-yn-1-yl)-2-(1H-1,2,4-triazol-1-yl)pyrimidine-4,6-diamine	Treatment of Huntington's disease	Palobiofarma S.L.	13 November 2014	16 December 2014
5-[8-methyl-9-(1-methylethyl)-2-(4-morpholinyl)-9H-purin-6-yl]-2-pyrimidinamine	Treatment of malignant mesothelioma	TMC Pharma Services Ltd	13 November 2014	16 December 2014
Adeno-associated viral vector serotype 10 carrying the human N-sulfoglucosamine sulfohydrolase cDNA	Treatment of mucopolysaccharidosis type IIIA (Sanfilippo A syndrome)	LYSOGENE	13 November 2014	16 December 2014
Adenovirus serotype 5 containing partial E1A deletion and an integrin-binding domain	Treatment of glioma	Alan Boyd Consultants Ltd	13 November 2014	16 December 2014
Allogeneic adipose-derived adult mesenchymal stem cells contained in a fibrin-based bioengineered dermis	Treatment of epidermolysis bullosa	Biodan Yelah S.L.	13 November 2014	16 December 2014

Active substance	Orphan indication	Sponsor	COMP opinion date	EC designation date
Allogeneic bone marrow derived mesenchymal cells expanded ex vivo in synthetic media	Prevention of graft-versus-host disease	Cell2B Advanced Therapeutics, SA	13 November 2014	16 December 2014
Allogeneic CD34+ cells expanded ex vivo with an aryl hydrocarbon receptor antagonist	Treatment of acute lymphoblastic leukaemia	Novartis Europharm Limited	13 November 2014	16 December 2014
Allogeneic ex vivo-generated natural killer cells from CD34+ umbilical cord blood progenitor cells	Treatment of acute myeloid leukaemia	IPD-Therapeutics BV	13 November 2014	16 December 2014
Amikacin sulfate	Treatment of *Pseudomonas aeruginosa* lung infection in cystic fibrosis	PlumeStars s.r.l.	13 November 2014	16 December 2014
Autologous collagen type II-specific regulatory T cells	Treatment of non-infectious uveitis	TxCell	13 November 2014	16 December 2014
Autologous T cells transduced with retroviral vector encoding an anti-CD19 CD28/CD3 zeta chimeric antigen receptor	Treatment of diffuse large B cell lymphoma	Kite Pharma UK, Ltd	13 November 2014	16 December 2014
Benserazide hydrochloride	Treatment of beta-thalassaemia intermedia and major	Isabelle Ramirez	13 November 2014	16 December 2014
Bevacizumab	Treatment of hereditary haemorrhagic telangiectasia	Dr Sophie Dupuis-Girod	13 November 2014	16 December 2014
Chenodeoxycholic acid	Treatment of inborn errors of primary bile acid synthesis	Sigma-Tau Pharma Ltd	13 November 2014	16 December 2014
Edaravone	Treatment of amyotrophic lateral sclerosis	Treeway B.V.	13 November 2014	16 December 2014
Exisulind	Treatment of familial cerebral cavernous malformations	Firc Institute of Molecular Oncology (IFOM)	13 November 2014	16 December 2014
Genetically modified serotype 5/3 adenovirus coding for granulocyte macrophage colony-stimulating factor	Treatment of malignant mesothelioma	Oncos Therapeutics Oy	13 November 2014	16 December 2014

Active substance	Orphan indication	Sponsor	COMP opinion date	EC designation date
Heat-killed *Mycobacterium obuense* (whole cell)	Treatment of pancreatic cancer	Immodulon Therapeutics Ltd	13 November 2014	16 December 2014
Pegylated recombinant human hyaluronidase PH20	Treatment of pancreatic cancer	Pharm. Research Associates (UK) Limited	13 November 2014	16 December 2014
Plerixafor	Treatment of WHIM syndrome	Groupe d'étude des neutropénies	13 November 2014	16 December 2014
Riluzole	Treatment of traumatic spinal cord injury	Dr Laurent Vinay	13 November 2014	16 December 2014
Single-chain urokinase plasminogen activator	Treatment of pleural empyema	Coté Orphan Consulting UK Limited	13 November 2014	16 December 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 December 2014 COMP monthly report

Active substance	Designated orphan indication	Sponsor/applicant	EU designation number
None			