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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

September 2015

The Committee for Orphan Medicinal Products held its 170th plenary meeting on 1-3 September 2015.

Orphan medicinal product designation

Positive opinions

The COMP adopted 20 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:

- Autologous T cells transduced with retroviral vector encoding an anti-CD19 CD28/CD3-zeta chimeric antigen receptor for treatment of mantle cell lymphoma, Kite Pharma UK, Ltd
- Autologous T cells transduced with retroviral vector encoding an anti-CD19 CD28/CD3-zeta chimeric antigen receptor for treatment of primary mediastinal large B-cell lymphoma, Kite Pharma UK, Ltd
- Nimodipine for treatment of aneurysmal subarachnoid haemorrhage, Dr Stefan Blesse
- Ovine-specific immunoglobulin (Fab) fragments raised against *Vipera berus* venom for treatment of snakebite envenomation, MicroPharm Limited
- Recombinant human IgG1 kappa light chain monoclonal antibody targeting plasma kallikrein for treatment of hereditary angioedema, Dyax Ltd
- Synthetic hepcidin for treatment of beta thalassaemia intermedia and major, Emas Pharma Ltd
- Synthetic peptide L-cysteine, L-cysteinylglycyl-L-glutaminyl-L-arginyl-L-.alpha.-glutamyl-L-threonyl-L-prolyl-L-.alpha.-glutamylglycyl-L-alanyl-L-.alpha.-glutamyl-L-alanyl-L-lysyl-L-prolyl-L-tryptophyl-L-tyrosyl-, cyclic (1.fwdarw.17)-disulfide for treatment of primary graft dysfunction following lung transplantation, Apeptico Forschung und Entwicklung GmbH

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2. Opinions adopted at the first COMP discussion:

- 2-(2-chlorophenyl)-4-[3-(dimethylamino)phenyl]-5-methyl-1H-pyrazolo[4,3-C]pyridine-3,6(2H,5H)-dione for treatment of systemic sclerosis, GenKyoTex Innovation S.A.S
- 2-chloro-N6-(3-iodobenzyl) adenosine-5'-N-methyluronamide for treatment of hepatocellular carcinoma, PBS Regulatory Consulting Group Limited
- 3-pentylbenzeneacetic acid sodium salt for treatment of idiopathic pulmonary fibrosis, ProMetic BioTherapeutics Ltd
- A highly purified formulation of *Staphylococcus aureus* protein A for treatment of immune thrombocytopenia, Coté Orphan Consulting UK Limited
- Ataluren for treatment of aniridia, PTC Therapeutics International Limited
- Dronabinol and cannabidiol for treatment of glioma, GW Research Ltd
- Mazindol for treatment of narcolepsy, NeuroLifeSciences
- N-(2-((4Z,7Z,10Z,13Z,16Z,19Z)-docosa-4,7,10,13,16,19-hexaenamido)ethyl)-2-hydroxybenzamide for treatment of Duchenne muscular dystrophy, FGK Representative Service GmbH
- Recombinant adeno-associated viral vector expressing the human *CNGA3* gene for treatment of achromatopsia caused by mutations in the *CNGA3* gene, TMC Pharma Services Ltd
- Recombinant human interleukin-3 truncated diphtheria toxin fusion protein for treatment of acute myeloid leukaemia, Spector Consulting SAS
- Sirolimus for treatment of tuberous sclerosis, Desitin Arzneimittel GmbH
- Three chimeric human/murine monoclonal antibodies against the Ebola (Zaire) surface glycoprotein for treatment for Ebola virus disease, Dr Stefan Blesse

3. Opinion adopted after appeal:

- Autologous human peripheral blood Vdelta1+ T lymphocytes activated in vitro by cytokine and monoclonal antibody treatment for treatment of chronic lymphocytic leukaemia / small lymphocytic lymphoma, Lymphact - Lymphocyte Activation Technologies S.A.

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 13 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

10 oral hearings took place.

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

Withdrawals of applications for orphan medicinal product designation

The COMP noted that 3 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.

Positive opinions on amendment of existing orphan drug designations

Details of those designated orphan medicinal products are provided in Annex 4.

Public summaries of opinions will be revised and re-published on the EMA website following adoption of the respective decisions on amendment by the European Commission.

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 products be kept in the EU registry of orphan medicinal products:

- Cresemba (isavuconazole) for treatment of invasive aspergillosis, Basilea Medical Ltd (EU/3/14/1284)
- Cresemba (isavuconazole) for treatment of mucormycosis, Basilea Medical Ltd (EU/3/14/1276)

Other matters

The main topics addressed during the meeting related to:

- Protocol assistance advice

Upcoming meetings

- The 171st meeting of the COMP will be held on 6-8 October 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	171	199	136 (68%)	62 (31%)	1 (1%)	138	6	7
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	**2298**	**2167**	**1566 (72%)**	**580 (27%)**	**21 (1%)**	**1544**	**106**	**114**

[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 July 2015 COMP monthly report

Active substance	Orphan indication	Sponsor	COMP opinion date	EC designation date
(S)-6-hydroxy-2,5,7,8-tetramethyl-N-((R)-piperidin-3-yl)chroman-2-carboxamide hydrochloride	Treatment of mitochondrial encephalomyopathy, lactic acidosis, and stroke-like episodes	Khondrion BV	16 July 2015	10 August 2015
2-((3-((4-((3-aminopropyl)amino)butyl)amino)propyl)amino)-N-((5S,5aS,8aR,9R)-9-(4-hydroxy-3,5-dimethoxyphenyl)-8-oxo-5,5a,6,8,8a,9-hexahydrofuro[3',4':6,7]naphtho[2,3-d][1,3]dioxol-5-yl)acetamide, tetrahydrochloride	Treatment of acute myeloid leukaemia	Pierre Fabre Médicament	18 June 2015	28 July 2015
2-(2-phenylvinyl)-4-[4-methylpiperazin-1-yl)]-6-(5-methyl-2H-pyrazol-3-yl-amino)-pyrimidine L(+) tartrate salt	Treatment of hepatocellular carcinoma	Dr Ulrich Granzer	16 July 2015	10 August 2015
2'-deoxyguanosylyl-(3',5'-phosphoryl)-2'-deoxythymidylyl-(3',5'-phosphoryl)- 2'-deoxyguanosylyl-(3',5'-phosphoryl)-2'-deoxycytidylyl-(3',5'-phosphoryl)-2'-deoxycytidylyl-(3',5'-phosphoryl)-2'-deoxycytidylyl-(3',5'-phosphoryl)-2'-deoxyguanosylyl-(3',5'-phosphoryl)-2'-deoxycytidilyl-(3',5'-phosphoryl)-2'-deoxycytidylyl-(3',5'-phosphoryl)-2'-deoxycytidylyl-(3',5'-phosphoryl)-2'-deoxycytidylyl-(3',5'-phosphoryl)-2'-deoxythymidylyl-(3',5'-phosphoryl)-2'-	Treatment of diffuse large B-cell lymphoma	PhaRA bvba	16 July 2015	10 August 2015

Active substance	Orphan indication	Sponsor	COMP opinion date	EC designation date
deoxyadenosyl-(3',5'-phosphoryl)-2'-deoxycytidylyl-(3',5'-phosphoryl)-2'-deoxyguanosylyl-(3',5'-phosphoryl)-2'-deoxycytidylyl-(3',5'-phosphoryl)-2'-deoxyguanosylyl-(3',5'-phosphoryl)-2'-deoxycytidylyl-(3',5'-phosphoryl)-2'-deoxyadenosyl-(3',5'-phosphoryl)-2'-deoxycytidylyl-(3',5'-phosphoryl)-2'-deoxyguanosylyl-2'-deoxycytidylyl-(3',5'-phosphoryl)-2'-deoxyadenosyl-(3',5'-phosphoryl)-2'-deoxycytidine, sodium salt				
Adeno-associated viral vector serotype 8 containing the human *MTM1* gene	Treatment of X-linked myotubular myopathy	Audentes Therapeutics UK Limited	16 July 2015	10 August 2015
Adeno-associated viral vector serotype 9 containing the human iduronate-2-sulfatase gene	Treatment of mucopolysaccharidosis type II (Hunter's syndrome)	Laboratorios del Dr. Esteve, S.A.	16 July 2015	10 August 2015
Adenovirus-associated viral vector serotype 2 containing the human *RPE65* gene	Treatment of retinitis pigmentosa	Alan Boyd Consultants Ltd	18 June 2015	28 July 2015
Allogeneic human adult stem cells, isolated from skeletal muscle and expanded ex vivo	Treatment of Duchenne muscular dystrophy	Karl Rouger	18 June 2015	28 July 2015
Allogeneic umbilical cord blood cells treated ex vivo with 16,16-dimethyl prostaglandin E2	Treatment of acute lymphoblastic leukaemia	Fate Therapeutics, LTD	16 July 2015	10 August 2015
Anti-H5N1 equine immunoglobulin $F(ab')_2$ fragments	Treatment of avian influenza	Fab'entech	18 June 2015	28 July 2015
Artesunate	Treatment of malaria	Dr Ulrich Granzer	18 June 2015	28 July 2015
Beloranib	Treatment of craniopharyngioma	Dr Ulrich Granzer	18 June 2015	28 July 2015
Cannabidiol	Treatment of perinatal asphyxia	GW Pharma Ltd	18 June 2015	28 July 2015

Active substance	Orphan indication	Sponsor	COMP opinion date	EC designation date
CD33-directed antibody-drug conjugate consisting of an antibody conjugated to a DNA cross-linking pyrrolobenzodiazepine dimer drug	Treatment of acute myeloid leukaemia	Seattle Genetics UK, Limited	16 July 2015	10 August 2015
Doxorubicin	Treatment of hepatoblastoma	Double Bond Pharmaceutical AB	18 June 2015	28 July 2015
Fibrinogen-coated albumin spheres	Treatment of acute radiation syndrome	Fibreu Limited	16 July 2015	10 August 2015
Fixed-dose combination of fosfomycin disodium and tobramycin	Treatment of cystic fibrosis	CURx Pharma (UK) Limited	16 July 2015	10 August 2015
Glycyl-L-2-methylprolyl-L-glutamic acid	Treatment of fragile X syndrome	QRC Consultants Ltd.	18 June 2015	28 July 2015
Glycyl-L-2-methylprolyl-L-glutamic acid	Treatment of Rett syndrome	QRC Consultants Ltd.	16 July 2015	10 August 2015
Human allogeneic bone-marrow-derived osteoblastic cells	Treatment of osteogenesis imperfecta	Bone Therapeutics SA	16 July 2015	10 August 2015
Human plasminogen	Treatment of plasminogen deficiency	ProMetic BioTherapeutics Ltd	18 June 2015	28 July 2015
Humanised IgG4 monoclonal antibody against extracellular tau	Treatment of progressive supranuclear palsy	Bristol-Myers Squibb Pharma EEIG	18 June 2015	28 July 2015
Hydrocinnamate-[Orn-Pro-dCha-Trp-Arg]acetate	Treatment of amyotrophic lateral sclerosis	PBS Regulatory Consulting Group Limited	18 June 2015	28 July 2015
Ibrutinib	Treatment of marginal zone lymphoma	Janssen-Cilag International N.V.	16 July 2015	10 August 2015
Inecalcitol	Treatment of acute myeloid leukaemia	Hybrigenics SA	18 June 2015	28 July 2015
Insulin human	Treatment of short bowel syndrome	Sirius Regulatory Consulting Limited	16 July 2015	10 August 2015
Lanreotide acetate	Treatment of autosomal dominant polycystic kidney disease	Prof. Dr. R.T.Gansevoort	18 June 2015	10 August 2015

Active substance	Orphan indication	Sponsor	COMP opinion date	EC designation date
Modified adenovirus serotype 5/35 containing a CMV promoter-driven transgene cassette with the human transgenes for a membrane-bound CD40 ligand and full length 4-1BBL	Treatment of pancreatic cancer	Lokon Pharma AB	18 June 2015	28 July 2015
Recombinant human acid ceramidase	Treatment of cystic fibrosis	Plexcera Therapeutics EU Limited	16 July 2015	10 August 2015
Sarizotan hydrochloride	Treatment of Rett syndrome	Newron Pharmaceuticals SpA	18 June 2015	28 July 2015
Synthetic double-stranded RNA oligonucleotide specific to hydroxyacid oxidase 1 gene	Treatment of primary hyperoxaluria type 1	Dicerna EU Limited	18 June 2015	28 July 2015
Synthetic hypericin	Treatment of cutaneous T-cell lymphoma	Kinesys Consulting Ltd	18 June 2015	28 July 2015
Triheptanoin	Treatment of carnitine palmitoyltransferase II deficiency	Ultragenyx UK Limited	18 June 2015	28 July 2015
Triheptanoin	Treatment of long-chain 3-hydroxyacyl-CoA dehydrogenase deficiency	Ultragenyx UK Limited	18 June 2015	28 July 2015
Triheptanoin	Treatment of mitochondrial trifunctional protein deficiency	Ultragenyx UK Limited	18 June 2015	28 July 2015
Verucerfont	Treatment of congenital adrenal hyperplasia	Neurocrine Therapeutics Ltd	16 July 2015	10 August 2015

Annex 3

Designated orphan medicinal products that have been subject of a new European Union marketing authorisation application under the centralised procedure since the July 2015 COMP monthly report

Active substance	Designated orphan indication	Sponsor/applicant	EU designation number
Cediranib	Treatment of ovarian cancer	AstraZeneca AB	EU/3/14/1303
Factor x	Treatment of hereditary factor X deficiency	BIO PRODUCTS LABORATORY	EU/3/07/471
Ixazomib	Treatment of systemic light chain amyloidosis	Takeda Pharma A/S	EU/3/12/1060

Annex 4

COMP opinions on amendment of existing orphan drug designations since July 2015 COMP monthly report

Active substance	Initial orphan indication	Amended orphan indication	Sponsor/applicant	EU designation number
Sialic acid	Treatment of hereditary inclusion body myopathy	Treatment of GNE myopathy	Ultragenyx UK Limited	