4 April 2016
EMA/COMP/150969/2016
Procedure Management and Committees Support Division

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

March 2016

The Committee for Orphan Medicinal Products held its 176th plenary meeting on 21-23 March 2016.

Orphan medicinal product designation

Positive opinions

The COMP adopted 18 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:

- Brincidofovir for prevention of cytomegalovirus disease, Chimerix UK Ltd;
- Human/murine chimeric monoclonal antibody against endoglin for treatment of soft tissue sarcoma, Tracon Pharma Limited;
- Tyr-Met-Phe-Pro-Asn-Ala-Pro-Tyr-Leu, Ser-Gly-Gln-Ala-Tyr-Met-Phe-Pro-Asn-Ala-Pro-Tyr-Leu-Pro-Ser-Cys-Leu-Glu-Ser, Arg-Ser-Asp-Glu-Leu-Val-Arg-His-His-Asn-Met-His-Gln-Arg-Asn-Met-Thr-Lys-Leu and Pro-Gly-Cys-Asn-Lys-Arg-Tyr-Phe-Lys-Leu-Ser-His-Leu-Gln-Met-His-Ser-Arg-Lys-His-Thr-Gly for treatment of acute myeloid leukaemia, SELLAS Life Sciences Group UK, Limited.

2. Opinions adopted at the first COMP discussion:

- (1E,6E)-1,7-bis(3,4-dimethoxyphenyl)-4-cyclobutylmethyl-1,6-heptadiene-3,5-dione for treatment of X-linked spinal and bulbar muscular atrophy (Kennedy's disease), Coté Orphan Consulting UK Limited;
- 2-methyl-1-[(4-[6-(trifluoromethyl)pyridin-2-yl]-6-{[2-(trifluoromethyl)pyridin-4-yl]amino}-1,3,5-triazin-2-yl)amino]propan-2-ol methanesulfonate for treatment of acute myeloid leukaemia, Celgene Europe Limited;

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- Antisense oligonucleotide complementary to the exonic splicer enhancer sequence atintron 26 of the centrosomal protein 290 pre-mRNA for treatment of Leber's congenital amaurosis, ProQR Therapeutics BV;
- Autologous dermal fibroblasts genetically modified ex vivo with a lentiviral vector containing the human *COL7A1* gene for treatment of epidermolysis bullosa, Intrexon Actobiotics N.V.;
- Autologous stromal vascular cell fraction from adipose tissue for treatment of systemic sclerosis, Cytori Ltd;
- Cannabidiol for prevention of graft-versus-host disease, Richardson Associates Regulatory Affairs Ltd;
- Combination of 4-hydroxyandrostenedione, *Serenoa serrulata* fruit extract and alpha lipoic acid for treatment of multiple symmetric lipomatosis, Dr Regenold GmbH Development·Regulatory·Market Access;
- Fluocinolone acetonide for treatment of non-infectious uveitis, Campharm Ltd;
- Humanised recombinant IgG4 anti-human tau antibody for treatment of progressive supranuclear palsy, Abbvie Ltd;
- N-carboxymethyl-glycyl-L-threonyl-L-histidyl-L-3,3-diphenylalanyl-L-piperidincarboxy-3-yl-L-arginyl-L-S-methylthio-cystyl-L-arginyl-L-tryptophyl-aminohexanyl-N-carboxamidomethyl-glycine N-hexadecylamide for treatment of beta thalassaemia intermedia and major, QRC Consultants Ltd;
- Recombinant adeno-associated viral vector serotype 9 carrying the gene for the human E6-AP ubiquitin protein ligase for treatment of Angelman syndrome, Voisin Consulting S.A.R.L.;
- Recombinant human cerebral dopamine neurotrophic factor for treatment of amyotrophic lateral sclerosis, Herantis Pharma Plc;
- Resiquimod for treatment of cutaneous T-cell lymphoma, Galderma R&D;
- S-acetyl-(S)-4'-phosphopantetheine, calcium salt for treatment of pantothenate-kinase-associated neurodegeneration, Acies Bio d.o.o.;
- Tyr-Met-Phe-Pro-Asn-Ala-Pro-Tyr-Leu, Ser-Gly-Gln-Ala-Tyr-Met-Phe-Pro-Asn-Ala-Pro-Tyr-Leu-Pro-Ser-Cys-Leu-Glu-Ser, Arg-Ser-Asp-Glu-Leu-Val-Arg-His-His-Asn-Met-His-Gln-Arg-Asn-Met-Thr-Lys-Leu and Pro-Gly-Cys-Asn-Lys-Arg-Tyr-Phe-Lys-Leu-Ser-His-Leu-Gln-Met-His-Ser-Arg-Lys-His-Thr-Gly for treatment of malignant mesothelioma, SELLAS Life Sciences Group UK, Limited.

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 5 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

6 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 5 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 products be kept in the EU registry of orphan medicinal products:

- Alprolix (eftrenonacog alfa) for treatment of haemophilia B, Biogen Idec Ltd (EU/3/07/453);
- Idelvion (albutrepenonacog alfa) for treatment of haemophilia B, CSL Behring GmbH (EU/3/09/723).

Review of the period of market exclusivity for orphan medicinal products

Article 8(2) of Regulation (EC) No 141/2000 of the European Parliament and the Council

In line with its responsibility to review whether a marketed orphan medicinal product still fulfils the designation criteria by the end of the fifth year following marketing authorisation upon request from a Member State, the COMP adopted 1 opinion recommending to the European Commission that the period of marketing exclusivity of the following orphan medicinal product be not reduced:

- Plenadren modified released tablets (hydrocortisone) for treatment of adrenal insufficiency, Shire Services BVBA (EU/3/06/372).

Other matters

The main topics addressed during the meeting related to:

- Protocol assistance advice

Upcoming meetings

- The 177th meeting of the COMP will be held on 19-21 April 2016.

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

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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[2]	Final negative COMP opinions	EC designations	Orphan medicinal products[3] authorised	Orphan designations included in authorised therapeutic indication
2016	81	67	49 (73%)	18 (27%)	0	40	1	1
2015	258	272	177 (65%)	94 (35%)	1 (1%)	190	14	21
2014	329	259	196 (76%)	62 (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%)	37 (40%)	1 (1%)	55	5	5
2002	80	75	43 (57%)	32 (42%)	2 (3%)	49	4	4
2001	83	90	62 (70%)	26 (29%)	1 (1%)	64	3	3
2000	72	32	26 (81%)	3 (10%)	0 (0%)	14	0	0
Total	**2466**	**2302**	**1656 (72%)**	**625 (27%)**	**21 (1%)**	**1636**	**115**	**129**

[2] Revision of the figures for 2015, 2014, 2003, 2002, 2001 and 2000
[3] 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 February 2016 COMP monthly report

Active substance	Orphan indication	Sponsor	COMP opinion date	EC designation date
Acalabrutinib	Treatment of chronic lymphocytic leukaemia / small lymphocytic lymphoma	Acerta Pharma, BV	18 February 2016	21 March 2016
Acalabrutinib	Treatment of lymphoplasmacytic lymphoma	Acerta Pharma, BV	18 February 2016	21 March 2016
Acalabrutinib	Treatment of mantle cell lymphoma	Acerta Pharma, BV	18 February 2016	21 March 2016
Adeno-associated viral vector serotype 5 containing a B-domain deleted variant of human coagulation factor VIII gene	Treatment of haemophilia A	BioMarin Europe Ltd	18 February 2016	21 March 2016
Adeno-associated viral vector serotype 8 encoding human ornithine transcarbamylase	Treatment of ornithine transcarbamylase deficiency	Pharma Gateway AB	18 February 2016	21 March 2016
Allogeneic Epstein-Barr virus specific cytotoxic T lymphocytes	Treatment of post-transplant lymphoproliferative disorder	Wainwright Associates Ltd	18 February 2016	21 March 2016
Diaspirin cross-linked haemoglobin	Treatment of oesophageal cancer	New B Innovation (UK) Limited	18 February 2016	21 March 2016
Exenatide	Treatment of idiopathic intracranial hypertension	Alan Boyd Consultants Ltd	18 February 2016	21 March 2016
Fenretinide	Treatment of cutaneous T-cell lymphoma	Clinipace GmbH	18 February 2016	21 March 2016
Florilglutamic acid (^{18}F)	Diagnosis of hepatocellular carcinoma	Piramal Imaging GmbH	18 February 2016	21 March 2016
Florilglutamic acid (^{18}F)	Diagnosis of glioma	Piramal Imaging GmbH	18 February 2016	21 March 2016

Active substance	Orphan indication	Sponsor	COMP opinion date	EC designation date
Fosbretabulin tromethamine	Treatment of gastro-entero-pancreatic neuroendocrine tumours	Diamond BioPharm Limited	18 February 2016	21 March 2016
Glucopyranosyl lipid A stable emulsion and recombinant New York esophageal squamous cell carcinoma-1 protein	Treatment of soft tissue sarcoma	Pharm Research Associates (UK) Limited	18 February 2016	21 March 2016
N-acetyl-D-mannosamine monohydrate	Treatment of GNE myopathy	Escala Therapeutics Ltd	18 February 2016	21 March 2016
Sindbis virus envelope pseudotyped lentiviral vector encoding New York esophageal squamous cell carcinoma-1 protein	Treatment of soft tissue sarcoma	Pharm Research Associates (UK)	18 February 2016	21 March 2016
Synthetic double-stranded siRNA oligonucleotide directed against hydroxyacid oxidase 1 mRNA and covalently linked to a ligand containing three N-acetylgalactosamine residues	Treatment of primary hyperoxaluria	Alnylam UK Limited	18 February 2016	21 March 2016
Ubenimex	Treatment of pulmonary arterial hypertension	Eiger Biopharmaceuticals Europe Limited	18 February 2016	21 March 2016

Annex 3

Designated orphan medicinal products that have been subject to a new European Union marketing authorisation application under the centralised procedure since the February 2016 COMP monthly report

Active substance	Designated orphan indication	Sponsor/applicant	EU designation number
Olaratumab	Treatment of soft tissue sarcoma	Eli Lilly Nederland B.V.	EU/3/15/1447
Paclitaxel	Treatment of ovarian cancer	Oasmia Pharmaceutical AB	EU/3/06/422
Pentosan polysulfate sodium	Treatment of interstitial cystitis	Bene-Arzneimittel GmbH	EU/3/14/1411