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Procedure Management and Committees Support Division

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

May 2016

The Committee for Orphan Medicinal Products held its 178th plenary meeting on 17-19 May 2016.

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:

- 2'-O-(2-methoxyethyl) phosphorothioate antisense oligonucleotide targeting the growth hormone receptor for treatment of acromegaly, Coté Orphan Consulting UK Limited;
- 3-(5-amino-2-methyl-4-oxoquinazolin-3(4H)-yl)piperidine-2,6-dione hydrochloride for treatment of diffuse large B-cell lymphoma, Celgene Europe Limited;
- Allogeneic donor-derived ex-vivo expanded T lymphocytes transduced with a retroviral vector containing inducible caspase 9 and truncated CD19 for treatment in haematopoietic stem cell transplantation, QRC Consultants Ltd;
- Citric acid monohydrate for treatment of acute liver failure, CATS Consultants GmbH;
- Donor T lymphocytes depleted ex vivo of host alloreactive T cells using photodynamic treatment for treatment in haematopoietic stem cell transplantation, Kiadis Pharma Netherlands B.V.;
- Eflornithine for treatment of glioma, Orbus Therapeutics Limited;
- Humanised anti-IL-6 receptor monoclonal antibody for treatment of neuromyelitis optica spectrum disorders, Chugai Pharma Europe Ltd;
- Humanised monoclonal antibody targeting interleukin-15 for treatment of eosinophilic oesophagitis, Dr Alain Vicari;

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- Melatonin for treatment of neonatal sepsis, Therapicon Srl;
- Melatonin for treatment of necrotising enterocolitis, Therapicon Srl;
- Molgramostim for treatment of acute respiratory distress syndrome, Serendex Pharmaceuticals A/S.

2. Opinions adopted at the first COMP discussion:

- Cyclocreatine for treatment of creatine deficiency syndromes, Pharma Gateway AB;
- Diclofenamide for treatment of periodic paralysis, Sun Pharmaceutical Industries Europe B.V.;
- Modified mRNA encoding the UGT1A1 protein for treatment of Crigler-Najjar syndrome, Alexion Europe SAS;
- Pyridoxine and L-pyroglutamic acid for treatment of fragile X syndrome, FGK Representative Service Ltd.;
- Recombinant humanised monoclonal IgG2 lambda antibody against human sclerostin for treatment of osteogenesis imperfecta, Mereo Biopharma Group Limited;
- Recombinant protein derived from the saliva of the *Ornithodoros moubata* tick for treatment of Guillain-Barré syndrome, Akari Therapeutics Plc;
- Setmelanotide for treatment of Prader-Willi syndrome, TMC Pharma Services Ltd;
- Teriparatide for treatment of hypoparathyroidism, Alacrita LLP.

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 10 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

11 oral hearings took place.

Withdrawals of applications for orphan medicinal product designation

The COMP noted that 1 application for orphan medicinal product designation was 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:

- Gazyvaro (obinutuzumab) for treatment of follicular lymphoma, Roche Registration Limited (EU/3/15/1504).

Other matters

The main topics addressed during the meeting related to:

- Protocol assistance advice

Upcoming meetings

- The 179th meeting of the COMP will be held on 14-16 June 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

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[2]	Final negative COMP opinions	EC designations	Orphan medicinal products[3] authorised	Orphan designations included in authorised therapeutic indication
2016	98	106	82 (77%)	24 (23%)	0	58	4	4
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	**2483**	**2341**	**1689 (72%)**	**631 (27%)**	**21 (1%)**	**1654**	**118**	**132**

[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 April 2016 COMP monthly report

Active substance	Orphan indication	Sponsor	COMP opinion date	EC designation date
(1E,6E)-1,7-bis(3,4-dimethoxyphenyl)-4-cyclobutylmethyl-1,6-heptadiene-3,5-dione	Treatment of X-linked spinal and bulbar muscular atrophy (Kennedy's disease)	Coté Orphan Consulting UK Limited	23 March 2016	28 April 2016
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	Treatment of acute myeloid leukaemia	Celgene Europe Limited	23 March 2016	28 April 2016
Antisense oligonucleotide complementary to the exonic splicer enhancer sequence atintron 26 of the centrosomal protein 290 pre-mRNA	Treatment of Leber's congenital amaurosis	ProQR Therapeutics BV	23 March 2016	28 April 2016
Autologous dermal fibroblasts genetically modified ex vivo with a lentiviral vector containing the human COL7A1 gene	Treatment of epidermolysis bullosa	Intrexon Actobiotics N.V.	23 March 2016	28 April 2016
Autologous stromal vascular cell fraction from adipose tissue	Treatment of systemic sclerosis	Cytori Ltd	23 March 2016	28 April 2016
Brincidofovir	Prevention of cytomegalovirus disease	Chimerix UK Ltd	23 March 2016	28 April 2016
Cannabidiol	Prevention of graft-versus-host disease	Richardson Associates Regulatory Affairs Ltd	23 March 2016	28 April 2016
Combination of 4-hydroxyandrostenedione, Serenoa serrulata fruit extract and alpha lipoic acid	Treatment of multiple symmetric lipomatosis	Dr Regenold GmbH Development·Regulatory·Market Access	23 March 2016	28 April 2016
Fluocinolone acetonide	Treatment of non-infectious uveitis	Campharm Ltd	23 March 2016	28 April 2016

Active substance	Orphan indication	Sponsor	COMP opinion date	EC designation date
Humanised recombinant IgG4 anti-human tau antibody	Treatment of progressive supranuclear palsy	Abbvie Ltd	23 March 2016	28 April 2016
Human/murine chimeric monoclonal antibody against endoglin	Treatment of soft tissue sarcoma	Tracon Pharma Limited	23 March 2016	28 April 2016
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	Treatment of beta thalassaemia intermedia and major	QRC Consultants Ltd	23 March 2016	28 April 2016
Recombinant adeno-associated viral vector serotype 9 carrying the gene for the human E6-AP ubiquitin protein ligase	Treatment of Angelman syndrome	Voisin Consulting S.A.R.L.	23 March 2016	28 April 2016
Recombinant human cerebral dopamine neurotrophic factor	Treatment of amyotrophic lateral sclerosis	Herantis Pharma Plc	23 March 2016	28 April 2016
Resiquimod	Treatment of cutaneous T-cell lymphoma	Galderma R&D	23 March 2016	28 April 2016
S-acetyl-(S)-4'-phosphopantetheine, calcium salt	Treatment of pantothenate-kinase-associated neurodegeneration	Acies Bio d.o.o.	23 March 2016	28 April 2016
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	Treatment of acute myeloid leukaemia	SELLAS Life Sciences Group UK, Limited	23 March 2016	28 April 2016
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-	Treatment of malignant mesothelioma	SELLAS Life Sciences Group UK, Limited	23 March 2016	28 April 2016

Active substance	Orphan indication	Sponsor	COMP opinion date	EC designation date
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				

Annex 3

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

Active substance	Designated orphan indication	Sponsor/applicant	EU designation number
Pacritinib	a) Treatment of post-essential thrombocythemia myelofibrosis b) Treatment of post-polycythemia vera myelofibrosis c) Treatment of primary myelofibrosis	Baxalta Innovations GmbH	EU/3/10/767 EU/3/10/769 EU/3/10/768
Tivozanib hydrochloride monohydrate	Treatment of renal cell carcinoma	EUSA PHARMA	EU/3/10/747