16 January 2017
EMA/30888/2017
Inspections, Human Medicines Pharmacovigilance and Committees

Committee for Orphan Medicinal Products (COMP)

Draft agenda for the meeting on 17-19 January 2017

Chair: Bruno Sepodes – Vice-Chair: Lesley Greene

17 January 2017, 09:00-19:30, room 2F

18 January 2017, 08:30-19:30, room 2F

19 January 2017, 08:30-15:30, room 2F

Health and safety information

In accordance with the Agency's health and safety policy, delegates are to be briefed on health, safety and emergency information and procedures prior to the start of the meeting.

Disclaimers

Some of the information contained in this agenda is considered commercially confidential or sensitive and therefore not disclosed. With regard to intended therapeutic indications or procedure scopes listed against products, it must be noted that these may not reflect the full wording proposed by applicants and may also vary during the course of the review. Additional details on some of these procedures will be published in the COMP meeting reports once the procedures are finalised.

Of note, this agenda is a working document primarily designed for COMP members and the work the Committee undertakes.

Note on access to documents

Some documents mentioned in the agenda cannot be released at present following a request for access to documents within the framework of Regulation (EC) No 1049/2001 as they are subject to on-going procedures for which a final decision has not yet been adopted. They will become public when adopted or considered public according to the principles stated in the Agency policy on access to documents (EMA/127362/2006).

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1. Introduction

1.1. Welcome and declarations of interest of members and experts

Pre-meeting list of participants and restrictions in relation to declarations of interests applicable to the items of the agenda for the COMP plenary session to be held 17-19 January 2017. See January 2017 COMP minutes (to be published post February 2017 COMP meeting).

1.2. Adoption of agenda

COMP agenda for 17-19 January 2017.

1.3. Adoption of the minutes

COMP minutes for 06-08 December 2016.

2. Applications for orphan medicinal product designation

2.1. For opinion

2.1.1. - EMA/OD/233/16

Treatment of Lennox-Gastaut syndrome

Action: For adoption, Oral explanation to be held on 17 January 2017 at 09:30

Documents tabled:
Draft Summary report with response to LoQs

Notes:
There has been 1 designation for this condition: EMEA/OD/047/04 Rufinamide

2.1.2. - EMA/OD/229/16

Treatment of diffuse large B cell lymphoma

Action: For adoption, Oral explanation to be held on 17 January 2017 at 11:00

Documents tabled:
Draft Summary report with response to LoQs

Notes:
There have been 3 designations for this condition: EMEA/OD/097/06 Enzastaurin hydrochloride , EMA/OD/071/14 (Z)-3-(3-(3,5-bis(trifluoromethyl)phenyl)-1H-1,2,4-triazol-1-yl)-N'-(pyrazin-2-yl)acrylohydrazide , EMA/OD/171/14 Autologous T cells transduced with retroviral vector encoding an anti-CD19 CD28/CD3 zeta chimeric antigen receptor

2.1.3. - EMA/OD/228/16

Treatment of gastric cancer

Action: For adoption, Oral explanation to be held on 17 January 2017 at 12:00

Documents tabled:
Draft Summary report with response to LoQs

Notes:
There have been 5 designations for this condition: EMEA/OD/056/02 G17(9) gastrin-Diphtheria Toxoid Conjugate , EMEA/OD/044/06 Catumaxomab , EMA/OD/083/10 Chimeric monoclonal antibody against claudin-18 splice variant 2 , EMA/OD/101/10 Tesetaxel , EMA/OD/012/14 Rilotumumab

Designations withdrawn: EMEA/OD/073/07 Tegafur, gimeracil, oteracil potassium , EMA/OD/022/11 Everolimus , EMA/OD/030/12 Ramucirumab

2.1.4. - EMA/OD/232/16

Treatment of glycogen storage disease type II (Pompe's disease)

Action: For adoption, Oral explanation to be held on 17 January 2017 at 15:00

Documents tabled:
Draft Summary report with response to LoQs

Notes:
There have been 2 designations for this condition: EMA/OD/057/11 Glycosylation independent lysosomal targeting tagged recombinant human acid alpha glucosidase, EMA/OD/098/16 Recombinant human acid alpha-glucosidase conjugated with mannose-6-phosphate analogues

2.1.5. - EMA/OD/245/16

Treatment of ovarian cancer

Action: For information

Documents tabled:
Withdrawal request of 15 December 2016

Notes:
Withdrawn
There have been 31 designations for this condition: EMEA/OD/019/02 Oregovomab, EMEA/OD/061/06 Paclitaxel (micellar) , EMEA/OD/080/03 Anti-epithelial cell adhesion molecule/anti-CD3 monoclonal antibody , EMEA/OD/044/03 Trabectedin , EMEA/OD/065/05 Imexon , EMEA/OD/063/07 Olaparib , EMEA/OD/110/07 Humanised monoclonal antibody to the folate receptor alpha , EMEA/OD/006/09 Human MHC non-restricted cytotoxic T-cell line , EMEA/OD/086/09 8-[4-(1-aminocyclobutyl)phenyl]-9-phenyl-1,2,4-triazolo[3,4-f][1,6]naphthyridin-3(2H)-one mono-hydrochloride , EMA/OD/015/10 (3S)-3-{4-[7-(aminocarbonyl)-2H-indazol-2-yl] phenyl} piperidine tosylate monohydrate salt , EMA/OD/021/10 Autologous dendritic cells pulsed with recombinant human-fusion protein (mucin 1 - glutathione S transferase) coupled to oxidised polymannose , EMA/OD/014/10 Pyr-His-Trp-Ser-Tyr-D-Lys(doxorubicinylglutarate)-Leu-Arg-Pro-Gly-NH2, acetate salt , EMA/OD/111/10 Veliparib , EMA/OD/054/11 20-pentaerythritol poly (oxy-1,2-ethanediyl)-carboxymethyl-glycinate-7-ethyl-10-hydroxycamptothecine 10-[1,4'-bipiperidine]-1'-carboxylate , EMA/OD/151/11 2-Allyl-1-[6-(1-hydroxy-1-methylethyl)pyridin-2-yl]-6-{[4-

(4-methylpiperazin-1-yl)phenyl]amino}-1,2-dihydro-3H-pyrazolo[3,4-d]pyrimidin-3-one , EMA/OD/085/12 rucaparib , EMA/OD/099/12 Lurbinectedin , EMA/OD/114/12 Alisertib , EMA/OD/147/12 Chimeric monoclonal antibody against claudin 6 , EMA/OD/039/13 Fosbretabulin tromethamine , EMA/OD/122/13 Trebananib , EMA/OD/186/13 Genetically modified serotype 5/3 adenovirus coding for granulocyte macrophage colony-stimulating factor , EMA/OD/059/14 Cediranib , EMA/OD/281/14 Humanised anti-folate receptor 1 monoclonal antibody conjugated to maytansinoid DM4 , EMA/OD/157/14 2-hydroxymethyl-2-methoxymethyl-1-azabicyclo[2,2,2]octan-3-one , EMA/OD/211/14 Chimeric group B adenovirus (11p/3) with deletions in the E3 and E4 regions , EMA/OD/223/14 N-methyl-4-({4-[({3-methyl(methylsulfonyl)amino]pyrazin-2- yl}methyl)amino]-5-(trifluoromethyl)pyrimidin-2-yl}amino)benzamide hydrochloride , EMA/OD/304/14 Human reovirus type 3 Dearing strain , EMA/OD/314/14 {2-amino-8-[4-(pyrrolidinylcarbonyl)phenyl]-(3H-benzo[f]azepin-4-yl)}-N,N-dipropylcarboxamide , EMA/OD/126/15 (5S,8S,10aR)-N-benzhydryl-5-((S)-2-(methylamino)propanamido)-3-(3-methylbutanoyl)-6-oxodecahydropyrrolo[1,2-a][1,5]diazocine-8-carboxamide , EMA/OD/159/16 Vaccine consisting of 5 survivin peptides with different human leukocyte antigen restrictions

Designations withdrawn: EMEA/OD/061/00 Human Milk Fat Globule 1 / Yttrium (90Y) human Milk Fat Globule 1 - S p isothiocyanatobenzyl-diethylenetriaminepentaacetic acid , EMEA/OD/062/01 Epothilone B, EMEA/OD/016/03 Murine anti-idiotypic antibody against OC125 antibody against CA125 antigen , EMEA/OD/071/09 Anti-EphA2 monoclonal antibody conjugated to maleimidocaproyl monomethylauristatin phenylalanine , EMA/OD/094/11 Vincaleukoblastin-23-oic acid, O4-deacetyl-2-[(2-mercaptoethoxy)carbonyl]hydrazide, disulfide with … , EMA/OD/002/12 1-(4-{4-amino-7-[1-(2-hydroxyethyl)-1H- pyrazol-4-yl] thieno [3,2-c]pyridin-3-yl}phenyl)-3-(3-fluorophenyl)urea

2.1.6. - EMA/OD/211/16

Treatment of glioma

Action: For information

Documents tabled:
Withdrawal request of 27 December 2016

Notes:
Withdrawn
There have been 42 designations for this condition: EMEA/OD/026/03 Herpes simplex virus lacking infected cell protein 34.5 , EMEA/OD/055/03 Gimatecan , EMEA/OD/050/04 Biotinylated anti-tenascin monoclonal antibody for use with 90-Yttrium , EMEA/OD/038/04 Anti epidermal growth factor receptor antibody h-R3 , EMEA/OD/030/05 Oligonucleotide phosphorothioate (TAAACGTTATAACGTTATGACGTCAT), sodium salt , EMEA/OD/068/05 Enzastaurin hydrochloride , EMEA/OD/110/05 4-[131I] iodo-L-phenylalanine , EMEA/OD/081/06 Autologous dendritic cells pulsed with autologous tumour cell lysate , EMEA/OD/050/07 Doxorubicin hydrochloride (drug eluting beads) , EMEA/OD/051/07 Irinotecan hydrochloride (drug eluting beads) , EMEA/OD/038/07 Iodine (131I) Chlorotoxin , EMEA/OD/004/08 Recombinant fusion protein of circulary-permuted IL-4 and pseudomonas exotoxin A, [IL-4(38-37)-PE38KDEL] , EMEA/OD/023/08 Topotecan hydrochloride (liposomal) , EMEA/OD/034/08 Gadodiamide (liposomal) , EMEA/OD/104/08 Autologous tumour-derived gp96 heat shock protein-peptide complex , EMEA/OD/098/09

Recombinant fusion protein consisting of the extracellular portion of CD95 fused to the Fc part of a human IgG1 molecule , EMA/OD/086/10 7-beta-hydroxycholesteryl-3-beta-oleate , EMA/OD/092/12 IL-12-secreting dendritic cells, loaded with autologous tumour lysate , EMA/OD/077/11 L-cysteine, L-leucyl-L-alpha-glutamyl-L-alpha-glutamyl-L-lysyl-L-lysylglycyl-L-asparaginyl-L-tyrosyl-L-valyl-L-valyl-L-threonyl-L-alpha-aspartyl-L-histidyl-S-[1-[(4-carboxycyclohexyl)methyl]-2,5-dioxo-3-pyrrolidinyl]-complex with keyhole limdinator_Applica, EMA/OD/050/11 2-hydroxyoleic acid , EMA/OD/157/11 Adenovirus-associated vector containing human Fas-c gene , EMA/OD/019/12 Doxorubicin (administered after synthetic double-stranded siRNA oligonucleotide directed against claudin-5 complexed with polyethyleneimine) , EMA/OD/170/12 4-[2-(6-methylpyridin-2-yl)-5,6-dihydro-4H-pyrrolo[1,2-b]pyrazol-3-yl]-quinoline-6-carboxamide monohydrate , EMA/OD/148/12 1,2:5,6-Dianhydrogalactitol , EMA/OD/136/12 Synthetic double-stranded siRNA oligonucleotide directed against Claudin-5 complexed with polyethyleneimine (prior to administration of doxorubicin) , EMA/OD/086/13 Autologous ex vivo expanded leukocytes treated with 5-aza-2'-deoxycytidine , EMA/OD/001/14 Autologous dendritic cells pulsed with RNA from glioma stem cells , EMA/OD/107/13 Allogeneic and autologous haptenised and irradiated cells and cell lysates derived from glioma , EMA/OD/174/13 Autologous dendritic cells pulsed with tumour antigen-derived synthetic peptides (MAGE-1, HER-2, AIM-2, TRP-2, gp-100, and interleukin-13 receptor alpha) , EMA/OD/111/14 Recombinant human bone morphogenetic protein 4 , EMA/OD/003/14 Paclitaxel-succinate- Arg-Arg-Leu-Ser-Tyr-Ser-Arg-Arg-Arg-Phe , EMA/OD/065/14 Humanised recombinant monoclonal antibody against epidermal growth factor receptor conjugated to maleimidocaproyl monomethylauristatin F , EMA/OD/132/14 Olaptesed pegol , EMA/OD/200/14 5,5'-(4-(trifluromethyl)benzylazanediyl)bis(methylene)diquinolin-8-ol , EMA/OD/159/14 Chloroquine , EMA/OD/176/14 Adenovirus serotype 5 containing partial E1A deletion and an integrin-binding domain , EMA/OD/251/14 Recombinant human glutamate oxaloacetate transaminase 1 , EMA/OD/206/15 N-(4-Methoxyphenyl)-N,2,6-trimethylfuro[2,3-d]pyrimidin-4-amine , EMA/OD/009/16 Eflornithine, EMA/OD/222/15 Delta-9-tetrahydrocannabinol and cannabidiol from extracts of the Cannabis sativa L. plant , EMA/OD/067/16 Zoledronic acid , EMA/OD/085/16 Temozolomide

Designations withdrawn: EMEA/OD/004/02 Pseudomonas exotoxin (domains II/III)-Interleukin 13 chimeric protein, EMEA/OD/074/01 Human transferrin conjugated to mutant diptheria toxin, EMEA/OD/067/01 Carmustine (solution for intratumoral injection), EMEA/OD/050/06 Iodine (131I) anti-tenascin monoclonal antibody 81C6 , EMEA/OD/037/02 Iodine (131I) anti-nucleohistone H1 chimeric biotinylated monoclonal antibody , EMEA/OD/067/03 Cilengitide , EMEA/OD/112/08 Talampanel , EMEA/OD/004/09 4,6,8-trihydroxy-10-(3,7,11-trimethyldodeca-2,6,10-trienyl)-5,10-dihydrodibenzo[b,e][1,4] diazepin-11-one , EMA/OD/031/10 Glutathione-pegylated liposomal doxorubicin hydrochloride , EMA/OD/049/12 Humanised monoclonal antibody against epidermal growth factor receptor , EMA/OD/113/15 Dronabinol and cannabidiol

2.1.7. - EMA/OD/249/16

Treatment of glioma

Action: For information

Documents tabled:
Withdrawal request of 27 December 2016

Notes:
Withdrawn
There have been 42 designations for this condition: Please see 2.1.6.

2.1.8. - EMA/OD/251/16

Treatment of sickle cell disease

Action: For adoption, Oral explanation to be held on 18 January 2017 at 10:00

Documents tabled:
Draft Summary report with response to LoQs

Notes:
There have been 12 designations for this condition: EMEA/OD/017/05 Extract of Sorghum bicolour leaf, Pterocarpus osun stem, Piper guineense seed and Caryophylli flower , EMEA/OD/107/08 2,2-dimethylbutyric acid, sodium salt , EMEA/OD/075/09 Pegylated carboxyhaemoglobin , EMA/OD/016/12 Levoglutamide , EMA/OD/040/12 Human Erythrocytes encapsulating Inositol Hexaphosphate , EMA/OD/026/12 Humanised monoclonal antibody targeting P-selectin , EMA/OD/162/12 Poloxamer 188 , EMA/OD/084/13 (1R,3R,4R,5S)-3-O-[2-O-benzoyl-3-O-(sodium(2S)-3-cyclohexyl-propanoate-2-yl)-β-D-galactopyranosyl]-4-O-(α-L-fucopyranosyl)-5-orothylamido-cyclohexane-1-carboxylic acid (ethyl-2-amidyl-ethyloxy-2-acetyl-(8-amino-1,3,6-naphthalene-tris sodium sulfonate) amide , EMA/OD/184/13 Autologous CD34+ haematopoietic stem cells transduced with lentiviral vector encoding the human beta A-T87Q-globin gene , EMA/OD/210/14 Sevufparin sodium , EMA/OD/187/16 2-hydroxy-6-((2-(1-isopropyl-1H-pyrazol-5-yl)pyridin-3-yl) methoxy)benzaldehyde , EMA/OD/144/16 Synthetic human hepcidin

Designation withdrawn: EMA/OD/249/14 5-hydroxymethyl-2-furfural

2.1.9. - EMA/OD/080/15

Treatment of fragile X syndrome

Action: For adoption, Oral explanation to be held on 18 January 2017 at 11:00

Documents tabled:
Draft Summary report with response to LoQs

Notes:
There have been 7 designations for this condition: EMA/OD/144/10 R-baclofen , EMA/OD/059/12 Mavoglurant , EMA/OD/105/14 (3S)-(+)-(5-chloro-2-methoxyphenyl)-1,3-dihydro-3-fluoro-6-(trifluoromethyl)-2H-indol-2-one , EMA/OD/137/14 Acamprosate calcium , EMA/OD/253/14 Tideglusib , EMA/OD/055/15 Glycyl-L-2-methylprolyl-L-glutamic acid , EMA/OD/034/16 Pyridoxine and L-pyroglutamic acid

2.1.10. - EMA/OD/244/16

Treatment of primary ciliary dyskinesia

Action: For adoption, Oral explanation to be held on 18 January 2017 at 12:00

Documents tabled:

Draft Summary report with response to LoQs

2.1.11. - EMA/OD/239/16

Treatment of pancreatic cancer

Action: For adoption, Oral explanation to be held on 18 January 2017 at 14:00

Documents tabled:
Draft Summary report with response to LoQs

Notes:
There have been 33 designations for this condition: EMEA/OD/055/02 G17(9) gastrin-Diphtheria Toxoid Conjugate , EMEA/OD/068/02 Rubitecan , EMEA/OD/009/05 Bovine bile extract , EMEA/OD/028/05 4-imino-1, 3-diazobicyclo-[3.1.0]-hexan-2-one , EMEA/OD/063/06 Paclitaxel (liposomal) , EMEA/OD/026/06 Human telomerase reverse transcriptase peptide (611-626) , EMEA/OD/103/06 Cisplatin (liposomal) , EMEA/OD/100/08 L-asparaginase encapsulated in erythrocytes , EMEA/OD/006/08 Nimotuzumab , EMEA/OD/080/08 Yttrium (90Y)-DOTA-radiolabelled humanized monoclonal antibody against mucin 1 , EMEA/OD/101/08 S-[2,3-bispalmitoyloxy-(2R)-propyl]-cysteinyl-GNNDESNISFKEK , EMEA/OD/030/09 Trabedersen , EMEA/OD/105/09 Brivudine , EMEA/OD/069/09 N-[(2S)-2,3-dihydroxypropyl]-3-[(2-fluoro-4-iodophenyl)amino]isonicotinamide hydrochloride , EMEA/OD/063/09 Masitinib mesilate , EMA/OD/135/10 Glufosfamide , EMA/OD/150/10 Salirasib , EMA/OD/007/11 Mixture of seven synthetic fragments consisting of p21 RAS peptides , EMA/OD/008/11 Genetically modified human adenovirus encoding human PH20 hyaluronidase , EMA/OD/051/11 Nanoliposomal irinotecan , EMA/OD/065/12 Mixture of two allogeneic human pancreatic cancer cell lines stably transduced with a retroviral vector encoding the murine alpha-(1,3)-galactosyltransferase gene , EMA/OD/037/13 (1-methyl-2-nitro-1H-imidazole-5-yl)methyl N,N'-bis(2-bromoethyl)diamidophosphate , EMA/OD/071/13 Chimeric monoclonal antibody against claudin-18 splice variant 2 , EMA/OD/164/13 Cysteamine bitartrate , EMA/OD/081/14 Immunoglobulin G1, anti-(human tumour-associated calcium signal transducer 2)(human-Mus musculus monoclonal hRS7 heavy chain), disulfide with human-Mus musculus monoclonal hRS7-chain, dimer, hexakis(thioether) with (4S)-4-[[[[4-[[(2S)-2-(4-aminobutyl)-2-[[2-[2-[[26-[4-[[[[4-[(3-mercapto-2,5-dioxo-1-pyrrolidinyl)methyl]cyclohexyl]carbonyl]amino]methyl]-1H-1,2,3-triazol-1-yl]-3,6,9,12,15,18,21,24-octaoxahexacos-1-yl]amino]-2-oxoethoxy]acetyl]amino]-1-oxoethyl]amino]phenyl]methoxy]carbonyl]oxy]-4,11-diethyl-9-hydroxy-1H-pyrano[3',4':6,7]indolizino[1,2-b]quinoline-3,14(4H,12H)-dione , EMA/OD/085/14 [5-Amino-1-(4-fluoro-phenyl)-1H-pyrazol-4-yl]-[3-(2,3-dihydroxy-propoxy)-phenyl]-methanone , EMA/OD/187/14 Herpes simplex type 1 virus containing cellular B-myb gene as tumour-specific promoter , EMA/OD/143/14 Heat-killed Mycobacterium obuense (whole cell) , EMA/OD/173/14 Pegylated recombinant human hyaluronidase PH20 , EMA/OD/302/14 Human reovirus type 3 Dearing strain , EMA/OD/034/15 Modified adenovirus serotype 5/35 containing a CMV promoter-driven transgene cassette with the human transgenes for a membrane-bound CD40 ligand (TMZ-CD40L) and full length 4-1BBL , EMA/OD/168/15 Live attenuated Listeria monocytogenes delta actA/delta inlB strain expressing human mesothelin , EMA/OD/169/15 Two allogenic irradiated pancreatic tumour cell lines

Designations withdrawn: EMEA/OD/070/02 Iodine (131I) Anti-CEA sheep-human chimeric monoclonal antibody , EMEA/OD/040/04 Deuterium oxide , EMEA/OD/097/05 26 base single stranded phosphodiester DNA oligonucleotide , EMEA/OD/111/07 Chimeric antibody to mesothelin , EMEA/OD/067/09 5'-O-(trans-9''-octadecenoyl)-1-beta-D-2'deoxy-2',2'-difluorocytidine , EMA/OD/087/10 Nanoparticle albumin-bound paclitaxel , EMA/OD/007/12 Polyinosine-polycytidylic acid coupled with the polycationic polyethyleneimine , EMA/OD/145/12 Recombinant human monoclonal antibody of the IgG1 kappa class against prostate stem cell antigen

2.1.12. - EMA/OD/224/16

Treatment of cystic fibrosis

Action: For adoption, Oral explanation to be held on 18 January 2017 at 15:00

Documents tabled:
Draft Summary report with response to LoQs

Notes:
There have been 37 designations for this condition: EMEA/OD/032/00 L-Lysine-N-Acetyl-L-Cysteinate, EMEA/OD/011/03 Recombinant dog gastric lipase , EMEA/OD/038/02 Duramycin, EMEA/OD/039/04 Dexamethasone sodium phosphate encapsulated in human erythrocytes , EMEA/OD/053/04 Alpha-1 antitrypsin (inhalation use) , EMEA/OD/107/04 3-[5-(2-fluoro-phenyl)-[1,2,4]oxadiazole-3-yl]-benzoic acid , EMEA/OD/062/05 Mannitolum , EMEA/OD/001/06 Heparin sodium , EMEA/OD/037/09 Ciprofloxacin (liposomal) , EMEA/OD/092/06 Ciprofloxacin (inhalation use) , EMEA/OD/104/06 Alginate oligosaccharide (G-block) fragment , EMEA/OD/041/07 Alpha1-proteinase inhibitor (inhalation use) , EMEA/OD/031/08 Avian polyclonal IgY antibody against Pseudomonas aeruginosa, EMEA/OD/010/08 N-(2,4-Di-tert-butyl-5-hydroxyphenyl)-1,4-dihydro-4-oxoquinoline-3-carboxamide , EMEA/OD/009/09 Hypothiocyanite / lactoferrin , EMA/OD/040/10 Nafamostat mesilate , EMA/OD/024/10 3-(6-(1-(2,2-difluorobenzo [d] [1,3] dioxol-5-yl)cyclopropanecarboxamido)-3-methylpyridin-2-yl)benzoic acid , EMA/OD/032/11 Sinapultide, dipalmitoylphosphatidylcholine palmitoyl-oleoyl phosphatidylglycerol, sodium salt and palmitic acid , EMA/OD/037/11 Multilamellar microvesicle comprising phosphatidylcholine, sphingomyelin, phosphatidylethanolamine, phosphatidylserine, phospatidylinositol and cholesterol , EMA/OD/046/11 Cysteamine , EMA/OD/058/12 Alpha-1 proteinase inhibitor (for inhalation use) , EMA/OD/005/13 Recombinant human CXCL8 mutant , EMA/OD/017/13 4,6,4'-trymethylangelicin , EMA/OD/096/13 Antisense oligonucleotide targeting the F508delta mutation of CFTR , EMA/OD/095/13 Nitric oxide , EMA/OD/159/13 Cysteamine , EMA/OD/156/13 11-(4-Dimethylamino-3-hydroxy-6-methyl-tetrahydro-pyran-2-yloxy)-2-ethyl-3,4,10-trihydroxy-3,5,6,8,10,12,14-heptamethyl-1-oxa-6-aza-cyclopentadecane-13,15-dione , EMA/OD/036/14 Nitric oxide , EMA/OD/013/14 Plasmid DNA encoding the human cystic fibrosis transmembrane conductance regulator gene complexed with a non-viral, cationic lipid based gene transfer agent , EMA/OD/002/14 1-(2,2-difluoro-1,3-benzodioxol-5-yl)-N-{1-[(2R)-2,3-dihydroxypropyl]-6-fluoro-2-(1-hydroxy-2-methylpropan-2-yl)-1Hindol-5-yl}cyclopropanecarboxamide , EMA/OD/131/14 4-[[(1S,4S)-5-[[4-[4-(Oxazol-2-yl)phenoxy]phenyl]methyl]-2,5-diazabicyclo[2.2.1]hept-2-yl]methyl]benzoic acid , EMA/OD/018/15 2-(7-ethoxy-4-(3-fluorophenyl)-1-oxophthalazin-2(1H)-yl)-N-methyl-N-(2-methylbenzo[d]oxazol-6-yl)acetamide , EMA/OD/319/14 Nitric oxide , EMA/OD/068/15 Fixed-dose combination of fosfomycin disodium and tobramycin , EMA/OD/061/15 Recombinant human acid ceramidase, EMA/OD/013/16 Sodium nitrite and

ethylenediaminetetraacetic acid , EMA/OD/100/16 (6aR, 10aR)-3-(1',1'-dimethylheptyl)-delta-8-tetrahydro-cannabinol-9-carboxylic acid

Designations withdrawn: EMEA/OD/009/02 Carbamic acid /[[4-[[3-[[4-[1-(4-hydroxyphenyl)-1-methyl-ethyl]phenoxy]methyl]phenyl]methoxy]-phenyl]iminomethyl]-,ethyl ester , EMEA/OD/064/00 8-cyclopentyl-1, 3-dipropylxanthine, EMEA/OD/018/03 Engineered protein inhibitor of human neutrophil elastase , EMEA/OD/075/02 Amiloride hydrochloride dihydrate , EMEA/OD/023/04 Recombinant human bile salt-stimulated lipase , EMEA/OD/054/05 Heparin sodium (inhalation use) , EMEA/OD/072/05 Denufosol tetrasodium , EMEA/OD/118/05 Glutathione , EMEA/OD/024/08 Levofloxacin hemihydrate , EMA/OD/032/14 Lumacaftor/ivacaftor

2.1.13. - EMA/OD/216/16

Treatment of ovarian cancer

Action: For information

Documents tabled:
Withdrawal request of 29 December 2016

Notes:
Withdrawn
There have been 31 designations for this condition: Please see 2.1.5.

2.1.14. - EMA/OD/246/16

Treatment of non-infectious uveitis

Action: For adoption, Oral explanation to be held on 18 January 2017 at 17:30

Documents tabled:
Draft Summary report with response to LoQs

Notes:
There have been 6 designations for this condition: EMA/OD/118/12 Voclosporin , EMA/OD/024/15 3-{[2,3,5,6-tetrafluoro-3'-(trifluoromethoxy)biphenyl-4-yl]carbamoyl}thiophene-2-carboxylic acid , EMA/OD/195/14 Autologous collagen type II-specific regulatory T cells , EMA/OD/320/14 Triamcinolone acetonide , EMA/OD/207/15 DNA plasmid encoding a recombinant fusion protein consisting of the extracellular domain of human TNFα p55 receptor linked to the human IgG1 Fc domain , EMA/OD/219/15 Fluocinolone acetonide

2.1.15. - EMA/OD/221/16

Treatment of Dravet syndrome

Action: For adoption, Oral explanation to be held on 18 January 2017 at 18:30

Documents tabled:
Draft Summary report with response to LoQs

Notes:

There have been 2 designations for this condition: EMA/OD/140/13 Fenfluramine hydrochloride, EMA/OD/083/14 Cannabidiol

2.1.16. - EMA/OD/252/16

Treatment of acetaminophen (paracetamol) overdose

Action: For adoption, Oral explanation to be held on 19 January 2017 at 09:00

Documents tabled:
Draft Summary report with response to LoQs

2.1.17. - EMA/OD/247/16

Treatment of bronchiolitis obliterans syndrome

Action: For adoption

Documents tabled:
Draft Summary report with response to LoQs

2.2. For discussion / preparation for an opinion

2.2.1. - EMA/OD/156/16

Treatment of cystic fibrosis

Action: For adoption

Documents tabled:
Draft Summary report

Notes:
There have been 37 designations for this condition: Please see 2.1.12.

2.2.2. - EMA/OD/285/16

Treatment of acute myeloid leukaemia

Action: For adoption

Documents tabled:
Draft Summary report

Notes:
There have been 48 designations for this condition: EMEA/OD/022/00 Gemtuzumab ozogamicin, EMEA/OD/028/04 Midostaurin , EMEA/OD/056/06 Antisense oligonucleotide 5'-d[P-Thio] (CCCTG CTCCC CCCTG GCTCC)-3' (see comments box for cenersen sodium) , EMEA/OD/098/04 Tipifarnib , EMEA/OD/094/04 Histamine dihydrochloride , EMEA/OD/066/05 1,2-bis(methylsulphonyl)-1-(2-chloroethyl)-2-[(methylamino)carbonyl]hydrazine , EMEA/OD/100/05 zosuquidar trihydrochloride , EMEA/OD/004/06 Decitabine , EMEA/OD/049/07 5'-O-(trans-9"-octadecenoyl)-1-ß-D-arabinofuranosyl cytosine , EMEA/OD/087/07 Recombinant human histone H1.3 and recombinant human N-bis-met-histone H1.3 , EMEA/OD/085/07 Azacitidine ,

EMEA/OD/099/07 N- (2-Amino-phenyl)-4-[(4-pyridin-3-yl-pyrimidin-2-ylamino)-methyl] benzamide , EMEA/OD/118/07 Ribonucleotide reductase R2 specific phosphorothioate oligonucleotide , EMEA/OD/015/08 Sapacitabine , EMEA/OD/048/08 Daunorubicin (liposomal) , EMEA/OD/105/08 N-(5-tert-Butylisoxazol-3-yl)-N'-{4-[7-(2-(morpholin-4-yl)ethoxy) imidazo[2,1-b][1,3]benzothiazol-2-yl]phenyl}urea di-hydrochloride salt , EMEA/OD/028/09 Tosedostat , EMEA/OD/091/09 1-Cyclopropyl-3-[3-(5-morpholin-4-ylmethyl-1H-benzoimidazol-2-yl)-1H-pyrazol-4-yl]-urea , EMEA/OD/147/09 2-methoxymethyl-2-hydroxymethyl-1-azabicyclo[2,2,2]octan-3-one , EMA/OD/044/10 Allogeneic T cells encoding an exogenous TK gene , EMA/OD/094/10 N-[(2S)-2,3-dihydroxypropyl]-3-[(2-fluoro-4-iodophenyl) amino] isonicotinamide hydrochloride , EMA/OD/161/10 Allogeneic bone marrow stem cells treated ex vivo with 16,16-dimethyl prostaglandin E2 , EMA/OD/101/11 Allogeneic human dendritic cells derived from a CD34+ progenitor cell line , EMA/OD/070/11 Liposomal combination of cytarabine and daunorubicin , EMA/OD/158/11 Vosaroxin , EMA/OD/167/12 L-asparaginase encapsulated in erythrocytes , EMA/OD/064/13 trans-N1-((1R,2S)-2-phenylcyclopropyl)cyclohexane-1,4-diamine bis-hydrochloride , EMA/OD/141/13 (2R,3R,4S,5R)-2-(6-amino-9H-purin-9-yl)-5-((((1r,3S)-3-(2-(5-(tert-butyl)-1Hbenzo[d]imidazol-2-yl)ethyl)cyclobutyl)(isopropyl) amino)methyl)tetrahydrofuran-3,4-diol , EMA/OD/181/13 Volasertib , EMA/OD/100/14 4-{[(2R,3S,4R,5S)-4-(4-Chloro-2-fluoro-phenyl)-3-(3-chloro-2-fluoro-phenyl)-4-cyano-5-(2,2-dimethyl-propyl)-pyrrolidine-2-carbonyl]-amino}-3-methoxy-benzoic acid , EMA/OD/061/14 (Z)-3-(3-(3,5-bis(trifluoromethyl)phenyl)-1H-1,2,4-triazol-1-yl)-N'-(pyrazin-2-yl)acrylohydrazide , EMA/OD/103/14 Donor T lymphocytes depleted ex vivo of host alloreactive T cells using photodynamic treatment , EMA/OD/175/14 Allogeneic ex vivo-generated natural killer cells from CD34+ umbilical cord blood progenitor cells , EMA/OD/240/14 Alvocidib , EMA/OD/188/14 Allogeneic, umbilical cord blood-derived, ex vivo-expanded, haematopoietic CD133+ cells / allogeneic, umbilical cord blood-derived, non-expanded, haematopoietic CD133- cells , EMA/OD/258/14 Ulocuplumab , EMA/OD/045/15 inecalcitol , EMA/OD/037/15 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 , EMA/OD/089/15 CD33-directed antibody-drug conjugate consisting of an antibody conjugated to a DNA cross-linking pyrrolobenzodiazepine dimer drug , EMA/OD/112/15 Recombinant human interleukin-3 truncated diphtheria toxin fusion protein , EMA/OD/145/15 Humanised monoclonal antibody of the IgG4 kappa isotype targeting CD47 , EMA/OD/165/15 Sodium (2R,3S,5R)-5-(4-amino-2-oxo-1,3,5-triazin-1(2H)-yl)-2-(hydroxymethyl)tetrahydrofuran-3-yl ((2R,3S,5R)-5-(2-amino-6-oxo-1H-purin-9(6H)-yl)-3-hydroxytetrahydrofuran-2-yl)methyl phosphate , EMA/OD/144/15 Combretastatin A1-diphosphate , EMA/OD/180/15 Arsenic trioxide , EMA/OD/205/15 Venetoclax , EMA/OD/233/15 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, EMA/OD/253/15 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 , EMA/OD/155/16 P-ethoxy growth factor receptor-bound protein 2 (Grb2) antisense oligonucleotide

Designations withdrawn: EMEA/OD/065/02 2-chloro-9-[2-deoxy-2-fluoro-ß-D-arabinofuranosyl]adenine , EMEA/OD/051/04 Homoharringtonine , EMEA/OD/059/04 Val-

Leu-Gln-Glu-Leu-Asn-Val-Thr-Val (Pr1 nanopeptide, sequence 169-177, of proteinase 3) , EMEA/OD/045/05 Troxacitabine , EMEA/OD/018/06 Human monoclonal antibody against inhibitory killer cell lg-like receptors (1-7 F9) , EMEA/OD/020/06 Lestaurtinib , EMEA/OD/024/07 Arsenic trioxide , EMEA/OD/069/07 Amonafide L-malate , EMEA/OD/060/08 2-[[3-({4-[(5-{2-[(3-Fluorophenyl)amino]-2-oxoethyl}-1H-pyrazol-3-yl)amino]-quinazolin-7-yl}oxy)propyl](ethyl)amino]ethyl dihydrogen phosphate trihydrate , EMEA/OD/118/08 Lintuzumab , EMEA/OD/090/08 Allogeneic ex vivo expanded umbilical cord blood cells , EMEA/OD/016/09 26 base single stranded phosphodiester DNA oligonucleotide , EMEA/OD/132/09 (1S, 2S, 3R, 4R)-3-(5-Fluoro-2-(3-methyl-4-(4-methylpiperazin-1-yl)-phenylamino)-pyrimidin-4-ylamino)-bicyclo[2.2.1]hept-5-ene-2-carboxamide benzoate) , EMA/OD/023/10 1-[2-(Benzo[1,2,5]thiadiazol-5-ylamino)-6-(2,6-dichloro-phenyl)-pyrido[2,3-d]pyrimidin-7-yl]-3-tert-butyl-urea , EMA/OD/156/10 Allogeneic umbilical cord blood cells treated ex vivo with 16,16-dimethyl prostaglandin E2 , EMA/OD/067/11 1-(4-{4-amino-7-[1-(2-hydroxyethyl)-1H- pyrazol-4-yl]thieno[3,2-c]pyridin-3-yl}phenyl)-3-(3-fluorophenyl)urea , EMA/OD/105/12 Liposomal daunorubicin

2.2.3. - EMA/OD/279/16

Treatment of Limb-Girdle muscular dystrophy type 2B

Action: For adoption

Documents tabled:
Draft Summary report

2.2.4. - EMA/OD/276/16

Treatment of Niemann-Pick disease type C

Action: For adoption

Documents tabled:
Draft Summary report

2.2.5. - EMA/OD/280/16

Treatment of retinitis pigmentosa

Action: For adoption

Documents tabled:
Draft Summary report

Notes:
There have been 19 designations for this condition: EMEA/OD/057/06 4,7,10,13,16,19-Docosahexaenoic acid , EMEA/OD/043/07 Adenovirus associated viral vector serotype 4 containing the human RPE65 gene , EMEA/OD/087/08 Recombinant human proinsulin , EMA/OD/162/10 9-cis-Retinyl acetate , EMA/OD/159/11 Encapsulated human retinal pigment epithelial cell line transfected with plasmid vector expressing human ciliary neurotrophic factor , EMA/OD/006/12 Recombinant human methionine proinsulin , EMA/OD/025/13 Expanded human allogeneic neural retinal progenitor cells extracted from neural retina , EMA/OD/015/13 Recombinant human nerve growth factor , EMA/OD/031/13 Adenovirus associated viral vector serotype 5 containing the human pde6β gene ,

EMA/OD/289/14 Sodium 3-[(4aR,6R,7R,7aS)-7-hydroxy-2-oxido-2-sulfanylidene-4a,6,7,7a-tetrahydro-4H-furo[3,2-d][1,3,2]dioxaphosphinin-6-yl]-2-bromo-6-phenyl-5H-imidazo[1,2-a]purin-9-one , EMA/OD/271/14 Myriocin , EMA/OD/327/14 Recombinant human mesencephalic astrocyte-derived neurotrophic factor , EMA/OD/040/15 Adenovirus-associated viral vector serotype 2 containing the human RPE65 gene , EMA/OD/158/16 Adeno-associated viral vector serotype 2/2 containing a gene encoding the channelrhodopsin-2 protein , EMA/OD/213/15 Allogeneic fetal human retinal progenitor cells expanded ex vivo , EMA/OD/208/15 4-[(2E)-1-oxo-3-(2,6,6-trimethyl-1-cyclohexen-1-yl)-2-propen-1-yl]-1-piperazinecarboxamide , EMA/OD/028/16 Adeno-associated viral vector serotype 2.7m8 containing the ChrimsonR-tdTomato gene , EMA/OD/102/16 Adenovirus associated viral vector serotype 5 containing the human RPGR gene , EMA/OD/146/16 Adeno-associated viral vector serotype 5 containing the human RLBP1 gene

Designations withdrawn: EMEA/OD/075/07 Recombinant human rod-derived cone viability factor , EMEA/OD/106/07 Allogeneic human umbilical cord tissue-derived cells , EMA/OD/021/12 17-(Dimethylaminoethylamino)-17-demethoxygeldanamycin (after administration of adeno-associated viral vector encoding an inducible short hairpin RNA targeting claudin-5) , EMA/OD/135/12 Adeno-associated viral vector encoding an inducible short hairpin RNA targeting claudin-5 (prior to administration of 17-dimethylaminoethylamino-17-demethocygeldanamycin) , EMA/OD/067/13 Unoprostone isopropyl

2.2.6. - EMA/OD/227/16

Treatment of epidermolysis bullosa

Action: For adoption

Documents tabled:
Draft Summary report

Notes:
There have been 11 designations for this condition: EMEA/OD/111/05 Bilayer engineered skin composed of keratinocytes from the patient (autologous) and fibroblasts from a donor (allogeneic) embedded in a plasma matrix , EMEA/OD/061/09 Allogeneic human dermal fibroblasts , EMA/OD/120/10 Dry extract from birch bark (DER 0.1-0.2:1), extraction solvent n-heptane 95% (V/V) , EMA/OD/145/13 Allantoin , EMA/OD/149/13 Diacerein , EMA/OD/201/13 Recombinant human alpha 1 chain homotrimer of type VII collagen , EMA/OD/197/14 Allogeneic adipose-derived adult mesenchymal stem cells contained in a fibrin-based bioengineered dermis , EMA/OD/218/15 Autologous dermal fibroblasts genetically modified ex vivo with a lentiviral vector containing the human COL7A1 gene , EMA/OD/299/14 Ex-vivo-expanded autologous human keratinocytes containing epidermal stem cells transduced with a COL17A1-encoding retroviral vector , EMA/OD/297/14 Ex-vivo-expanded autologous human keratinocytes containing epidermal stem cells transduced with a LAMB3-encoding retroviral vector , EMA/OD/188/15 Ex-vivo-expanded autologous fibroblasts transduced with lentiviral vector containing the COL7A1 gene

Designation withdrawn: EMA/OD/172/10 Human dermal fibroblasts cultured on a bioresorbable polyglactin mesh

2.2.7. - EMA/OD/274/16

Treatment of plasma cell myeloma

Action: For adoption

Documents tabled:
Draft Summary report

Notes:
There have been 8 designations for this condition: EMA/OD/072/13 Recombinant human monoclonal IgM antibody targeting glucose-regulated protein 78 , EMA/OD/038/13 Daratumumab , EMA/OD/198/13 humanized monoclonal antibody against CD38 , EMA/OD/035/14 Marizomib , EMA/OD/087/14 Selinexor , EMA/OD/293/14 Melphalan flufenamide , EMA/OD/214/14 Synthetic signal peptide of human Mucin-1 (amino acids 1-21) , EMA/OD/277/14 Reduced oxidised N-acetyl heparin

2.2.8. - EMA/OD/275/16

Treatment of Lennox-Gastaut syndrome

Action: For adoption

Documents tabled:
Draft Summary report

Notes:
There has been 1 designation for this condition: EMEA/OD/047/04 Rufinamide

2.2.9. - EMA/OD/284/16

Treatment of amyotrophic lateral sclerosis

Action: For adoption

Documents tabled:
Draft Summary report

Notes:
There have been 18 designations for this condition: EMEA/OD/053/06 Arimoclomol , EMEA/OD/102/07 Filgrastim , EMEA/OD/096/08 (6R)-4,5,6,7-tetrahydro-N6-propyl-2,6-benzothiazole-diamine dihydrochloride monohydrate , EMEA/OD/108/09 Recombinant human vascular endothelial growth factor , EMA/OD/043/11 Smilagenin , EMA/OD/106/11 S[+] apomorphine , EMA/OD/138/11 6-ethynyl-1-(pentan-3-yl)-1H-imidazo[4,5-b]pyrazin-2(3H)-one , EMA/OD/011/13 Autologous bone marrow-derived mesenchymal stromal cells secreting neurotrophic factors , EMA/OD/023/13 Sodium chlorite , EMA/OD/044/13 Allogeneic motor neuron progenitor cells derived from human embryonic stem cells , EMA/OD/184/14 Edaravone , EMA/OD/283/14 Enoxacin , EMA/OD/032/15 Edaravone , EMA/OD/051/15 Hydrocinnamate-[Orn-Pro-dCha-Trp-Arg]acetate , EMA/OD/011/16 H-Phe-Ser-Arg-Tyr-Ala-Arg-OH-acetate , EMA/OD/241/15 Recombinant human cerebral dopamine neurotrophic factor , EMA/OD/081/16 Masitinib mesilate , EMA/OD/120/16 Synthetic ribonucleic acid oligonucleotide directed against superoxide dismutase 1 messenger ribonucleic acid

Designations withdrawn: EMEA/OD/029/00 Xaliproden hydrochloride, EMEA/OD/030/06 Cholest-4-en-3-one, oxime, EMEA/OD/125/07 Sarsasapogenin , EMEA/OD/012/09 Talampanel , EMA/OD/060/10 Recombinant humanised monoclonal antibody to human Nogo-A protein of the IgG1/kappa class

2.2.10. - EMA/OD/278/16

Treatment of short bowel syndrome

Action: For adoption

Documents tabled:
Draft Summary report

Notes:
There have been 3 designations for this condition: EMEA/OD/045/01 [gly2]-recombinant human glucagon-like peptide, EMA/OD/080/14 Oxalobacter formigenes strain HC-1 , EMA/OD/050/15 Insulin human (rDNA)

2.2.11. - EMA/OD/283/16

Treatment of epidermolysis bullosa

Action: For adoption

Documents tabled:
Draft Summary report

Notes:
There have been 11 designations for this condition: Please see 2.2.6.

2.2.12. - EMA/OD/273/16

Treatment of pancreatic cancer

Action: For adoption

Documents tabled:
Draft Summary report

Notes:
There have been 33 designations for this condition: Please see 2.1.11.

2.2.13. - EMA/OD/272/16

Treatment of myelodysplastic syndromes

Action: For adoption

Documents tabled:
Draft Summary report

Notes:
There have been 6 designations for this condition: EMEA/OD/059/01 Azacitidine, EMEA/OD/059/02 Decitabine , EMEA/OD/083/03 3-(4'aminoisoindoline-1'-one)-1-piperidine-

2,6-dione , EMEA/OD/014/08 Sapacitabine , EMA/OD/161/11 (E)-2,4,6-trimethoxystyryl-3-carboxymethylamino-4-methoxybenzyl-sulfone sodium salt , EMA/OD/048/14 Recombinant fusion protein consisting of a modified form of the extracellular domain of human Activin Receptor IIB linked to the human IgG1 Fc domain

Designations withdrawn: EMEA/OD/047/00 Arsenic trioxide, EMEA/OD/117/08 Lintuzumab , EMEA/OD/033/09 Allogeneic ex vivo expanded umbilical cord blood cells

2.2.14. - EMA/OD/267/16

Treatment of Neuromyelitis Optica Spectrum Disorders (NMOSD)

Action: For adoption

Documents tabled:
Draft Summary report

2.2.15. - EMA/OD/271/16

Treatment of neuroblastoma

Action: For adoption

Documents tabled:
Draft Summary report

Notes:
There have been 9 designations for this condition: EMEA/OD/096/07 Iodine (131I) iobenguane , EMEA/OD/093/09 16-base single-stranded PNA oligonucleotide linked to a 7-aminoacid peptide , EMA/OD/126/10 Eflornithine , EMA/OD/002/11 Chimeric monoclonal antibody against GD2 , EMA/OD/020/12 16 base single stranded peptide nucleic acid oligonucleotide - 7 aminoacids peptide , EMA/OD/112/12 Chimeric monoclonal antibody against GD2 , EMA/OD/199/14 Chimeric monoclonal antibody specific to O-acetyl-GD2 antigen , EMA/OD/326/14 Sodium 2-hydroxylinoleate , EMA/OD/136/15 N-[5-(3,5-difluorobenzyl)-1H-indazol-3-yl]-4-(4 methylpiperazin-1-yl)-2-(tetrahydro-2H-pyran-4-ylamino)benzamide

Designation withdrawn: EMEA/OD/013/09 Murine monoclonal antibody to GD2

2.2.16. - EMA/OD/234/16

Treatment of granulosa cell tumors

Action: For adoption

Documents tabled:
Draft Summary report

2.2.17. - EMA/OD/236/16

Treatment of granulosa cell tumors

Action: For adoption

Documents tabled:

Draft Summary report

2.2.18. - EMA/OD/288/16

Treatment of short bowel syndrome

Action: For adoption

Documents tabled:
Draft Summary report

Notes:
There have been 3 designations for this condition: Please see 2.2.10.

2.2.19. - EMA/OD/289/16

Treatment of cystic fibrosis

Action: For adoption

Documents tabled:
Draft Summary report

Notes:
There have been 37 designations for this condition: Please see 2.1.12.

2.2.20. - EMA/OD/255/16

Treatment of pancreatic cancer

Action: For adoption

Documents tabled:
Draft Summary report

Notes:
There have been 33 designations for this condition: Please see 2.1.11.

2.2.21. - EMA/OD/217/16

Treatment of cystic fibrosis

Action: For adoption

Documents tabled:
Draft Summary report

Notes:
There have been 37 designations for this condition: Please see 2.1.12.

2.2.22. - EMA/OD/219/16

Treatment of invasive aspergillosis

Action: For adoption

Documents tabled:
Draft Summary report

Notes:
There have been 2 designations for this condition: EMA/OD/009/14 Isavuconazonium sulfate , EMA/OD/104/16 2-(1,5-dimethyl-3-phenyl-1H-pyrrol-2-yl)- N-{4-[4-(5-fluoro-pyrimidin-2-yl) piperazin- 1-yl]-phenyl}-2-oxo-acetamide

2.2.23. - EMA/OD/256/16

Treatment of Von Hippel Lindau Disease

Action: For adoption

Documents tabled:
Draft Summary report

2.2.24. - EMA/OD/262/16

Treatment of pancreatic cancer

Action: For adoption

Documents tabled:
Draft Summary report

Notes:
There have been 33 designations for this condition: Please see 2.1.11.

2.2.25. - EMA/OD/269/16

Treatment of achondroplasia

Action: For adoption

Documents tabled:
Draft Summary report

Notes:
There has been 1 designation for this condition: EMA/OD/149/12 Modified recombinant human C-type natriuretic peptide

2.2.26. - EMA/OD/261/16

Treatment of idiopathic pulmonary fibrosis

Action: For adoption

Documents tabled:
Draft Summary report

Notes:
There have been 12 designations for this condition: EMEA/OD/052/04 Pirfenidone , EMEA/OD/054/07 Interferon gamma , EMEA/OD/104/09 Macitentan , EMA/OD/079/10 2-(2-chlorophenyl)-4-[3-(dimethylamino)phenyl]-5-methyl-1H-pyrazolo[4,3-C]pyridine-

3,6(2H,5H)-dione , EMA/OD/091/11 4-[[9-[(3S)-tetrahydro-3-furanyl]-8-[(2,4,6-trifluorophenyl)amino]-9H-purin-2-yl]amino]-trans-cyclohexanol , EMA/OD/048/12 Recombinant human pentraxin-2 , EMA/OD/186/12 nintedanib , EMA/OD/051/14 Humanised anti-alpha v beta 6 monoclonal antibody , EMA/OD/130/14 1-(6-benzothiazolylsulfonyl)-5-chloro-1H-indole-2-butanoic acid , EMA/OD/072/15 3-pentylbenzeneacetic acid sodium salt , EMA/OD/046/16 3-[4-(1H-imidazol-1-ylmethyl)phenyl]-5-(2-methylpropyl)thiophene-2-[(N-butyloxylcarbamate)-sulphonamide] sodium salt , EMA/OD/088/16 2-((2-ethyl-6-(4-(2-(3-hydroxyazetidin-1-yl)-2-oxoethyl)-piperazin-1-yl)-8-methylimidazo[1,2-alpha]pyridin-3-yl)-(methyl)amino)-4-(4-fluorophenyl)-thiazole-5-carbonitrile

Designations withdrawn: EMEA/OD/002/05 Interferon gamma , EMEA/OD/033/04 Heparin-Sodium , EMEA/OD/075/04 Acetylcysteine , EMEA/OD/105/07 Recombinant human monoclonal antibody against transforming growth factor beta-1, 2 and 3 , EMEA/OD/027/08 Bosentan , EMA/OD/029/10 Ambrisentan , EMA/OD/111/12 Tralokinumab

2.2.27. - EMA/OD/266/16

Treatment of pancreatic cancer

Action: For adoption

Documents tabled:
Draft Summary report

Notes:
There have been 33 designations for this condition: Please see 2.1.11.

2.2.28. - EMA/OD/242/16

Treatment of amyotrophic lateral sclerosis

Action: For adoption

Documents tabled:
Draft Summary report

Notes:
There have been 18 designations for this condition: EMEA/OD/053/06 Arimoclomol , EMEA/OD/102/07 Filgrastim , EMEA/OD/096/08 (6R)-4,5,6,7-tetrahydro-N6-propyl-2,6-benzothiazole-diamine dihydrochloride monohydrate , EMEA/OD/108/09 Recombinant human vascular endothelial growth factor , EMA/OD/043/11 Smilagenin , EMA/OD/106/11 S[+] apomorphine , EMA/OD/138/11 6-ethynyl-1-(pentan-3-yl)-1H-imidazo[4,5-b]pyrazin-2(3H)-one , EMA/OD/011/13 Autologous bone marrow-derived mesenchymal stromal cells secreting neurotrophic factors , EMA/OD/023/13 Sodium chlorite , EMA/OD/044/13 Allogeneic motor neuron progenitor cells derived from human embryonic stem cells , EMA/OD/184/14 Edaravone , EMA/OD/283/14 Enoxacin , EMA/OD/032/15 Edaravone , EMA/OD/051/15 Hydrocinnamate-[Orn-Pro-dCha-Trp-Arg]acetate , EMA/OD/011/16 H-Phe-Ser-Arg-Tyr-Ala-Arg-OH-acetate , EMA/OD/241/15 Recombinant human cerebral dopamine neurotrophic factor , EMA/OD/081/16 Masitinib mesilate , EMA/OD/120/16 Synthetic ribonucleic acid oligonucleotide directed against superoxide dismutase 1 messenger ribonucleic acid

Designations withdrawn: EMEA/OD/029/00 Xaliproden hydrochloride, EMEA/OD/030/06 Cholest-4-en-3-one, oxime , EMEA/OD/125/07 Sarsasapogenin , EMEA/OD/012/09 Talampanel , EMA/OD/060/10 Recombinant humanised monoclonal antibody to human Nogo-A protein of the IgG1/kappa class

2.2.29. - EMA/OD/268/16

Treatment of hereditary haemorrhagic telangiectasia

Action: For adoption

Documents tabled:
Draft Summary report

Notes:
There have been 3 designations for this condition: EMEA/OD/138/09 Raloxifene hydrochloride, EMA/OD/144/14 Bazedoxifene acetate , EMA/OD/167/14 Bevacizumab

2.2.30. - EMA/OD/281/16

Treatment of Erdheim chester disease

Action: For adoption

Documents tabled:
Draft Summary report

2.3. Revision of the COMP opinions

None

2.4. Amendment of existing orphan designations

2.4.1. Synthetic double-stranded siRNA oligonucleotide directed against transthyretin Mrna – EMA/OD/142/10, EU/3/11/857

Alnylam UK Limited - United Kingdom; Treatment of familial amyloid polyneuropathy

Action: For adoption, Oral explanation to be held on 19 January 2017 at 10:00

Document tabled:
Amended draft Summary report

2.5. Appeal

None

2.6. Nominations

2.6.1. New applications for orphan medicinal product designation - Appointment of COMP coordinators

Action: For adoption

Document tabled:
OMPD applications - appointment of coord. at the 17-19 January 2017 COMP meeting

2.7. Evaluation on-going

Thirty three applications for orphan designation will not be discussed as evaluation is on-going.

Action: For information

Notes:
See 7.8.1. Table 6. Evaluation on-going.

3. Requests for protocol assistance with significant benefit question

3.1. Ongoing procedures

3.1.1. -

Treatment of paroxysmal nocturnal haemoglobinuria

Action: For discussion/adoption

Documents tabled:
First reports

3.1.2. -

Treatment of autosomal dominant polycystic kidney disease

Action: For discussion/adoption

Documents tabled:
First reports

3.2. Finalised letters

None

3.3. New requests

3.3.1. -

Treatment of Gaucher disease

Action: For information

3.3.2. -

Treatment of amyotrophic lateral sclerosis

Action: For information

3.3.3. -

Treatment of narcolepsy

Action: For information

3.3.4. -

Treatment of Langerhans cell histiocytosis

Action: For information

3.3.5. -

Treatment of glioma

Action: For information

4. Review of orphan designation for orphan medicinal products at time of initial marketing authorisation

4.1. Orphan designated products for which CHMP opinions have been adopted

4.1.1. Ledaga - chlormethine – EMA/OD/112/11, EU/3/12/963, EMEA/H/C/002826

Actelion Registration Ltd.; Treatment of cutaneous T-cell lymphoma

Action: For discussion

Document(s) tabled:
Draft report on review of OMPD
CHMP assessment report

Notes:
Status of the procedure at the CHMP: CHMP opinion adopted in December 2016

4.2. Orphan designated products for discussion prior to adoption of CHMP opinion

4.2.1. - expanded human allogeneic mesenchymal adult stem cells extracted from adipose tissue – EMEA/OD/054/09, EU/3/09/667, EMEA/H/C/004258

TIGENIX, S.A.U.; Treatment of anal fistula

Action: For discussion

Document(s) tabled:
Draft report on review of OMPD
CHMP assessment report

4.2.2. - parathyroid hormone – EMA/OD/102/13, EU/3/13/1210, EMEA/H/C/003861

NPS Pharma Holdings Limited; Treatment of hypoparathyroidism

Action: For discussion

Document(s) tabled:
Draft report on review of OMPD
CHMP assessment report

4.2.3. - pentosan polysulfate sodium – EMA/OD/179/14, EU/3/14/1411, EMEA/H/C/004246

Bene-Arzneimittel GmbH; Treatment of interstitial cystitis

Action: For discussion

Document(s) tabled:
Draft report on review of OMPD
CHMP assessment report

4.2.4. Raxone – idebenone - type II variation – EMEA/OD/077/06, EU/3/07/437, EMEA/H/C/003834/II/0003

Santhera Pharmaceuticals (Deutschland) GmbH; Treatment of Duchenne muscular dystrophy

Action: For discussion

Document(s) tabled:
Draft report on review of OMPD
CHMP assessment report

4.2.5. – nusinersen - EMEA/H/C/004312, EMA/OD/141/11, EU/3/12/976

Biogen Idec Ltd; Treatment of 5q spinal muscular atrophy

Action: For discussion

Document(s) tabled:
Draft report on review of OMPD

4.3. Appeal

4.3.1. Chenodeoxycholic acid sigma-tau - chenodeoxycholic acid – EMA/OD/196/14, EU/3/14/1406, EMEA/H/C/004061

Sigma-tau Arzneimittel GmbH; Treatment of inborn errors of primary bile acid synthesis

Action: For adoption, Oral explanation to be held on 17 January 2017 at time 14:00

Documents tabled:
Draft report on review of OMPD
CHMP assessment report

Notes:
Status of the procedure at the CHMP: Opinion adopted in September 2016.
Appeal of the negative COMP opinion adopted in October 2016.

4.4. On-going procedures

Action: For information

4.5. Public Summary of Opinions

Action: For information

5. Review of orphan designation for authorised orphan medicinal products at time marketing authorisation extension

5.1. After adoption of CHMP opinion

None

5.2. Prior to adoption of CHMP opinion

5.2.1. Nplate - recombinant megakaryopoiesis-stimulating protein – EMEA/OD/008/05, EU/3/05/283

Amgen Europe BV - The Netherlands; Treatment of idiopathic thrombocytopenic purpura

COMP coordinator: To be appointed

Action: For discussion/appointment of coordinators

Document(s) tabled:
Sponsor's report

5.2.2. Soliris – eculizumab - EMA/OD/062/14; EU/3/14/1304

Alexion Europe SAS; Treatment of myasthenia gravis

COMP coordinator: To be appointed

Action: For appointment of coordinators

5.3. Appeal

None

6. Application of Article 8(2) of the Orphan Regulation

None

7. Organisational, regulatory and methodological matters

7.1. Mandate and organisation of the COMP

7.1.1. Protocol Assistance Working Group

Proposed meeting time on 19 January 2017 at time 13:00, function room

Document(s) tabled:
Pending

7.1.2. Recommendations on eligibility to PRIME – report from CHMP

Action: For information

Document(s) tabled:
PRIME eligibility requests - list of adopted outcomes December 2016

7.1.3. Timing of COMP vs CHMP opinions

An update on measures to be taken to meet EC requirements to speed up the timing between CHMP and COMP opinions

Action: For information

7.2. Coordination with EMA Scientific Committees or CMDh-v

7.2.1. PDCO/COMP Working Group

Proposed meeting time on 18 January 2017 at time 13:00, room 8C

7.3. Coordination with EMA Working Parties/Working Groups/Drafting Groups

7.3.1. Working Party with Patients' and Consumers' Organisations (PCWP)

Training session for patients and consumers interested in EMA activities - 29 November 2016

PCWP meeting with all eligible organisations – 30 November 2016

Action: For information

Document(s) tabled:
Agenda of the Training session for patients and consumers interested in EMA activities - 29 Nov (EMA/636824/2016)
Agenda of the PCWP meeting with all eligible organisations – 30 Nov (EMA/668397/2016)

PCWP Work plan 2017

Action: For adoption

Document(s) tabled:
PCWP Work plan 2017 (EMA/540720/2016)

7.3.2. Working Party with Healthcare Professionals' Organisations (HCPWP)

HCPWP Work plan 2017

Action: For adoption

Document(s) tabled:
HCPWP Work plan 2017 (EMA/493549)

7.3.3. Working Party with Patients' and Consumers' Organisations (PCWP) and Healthcare Professionals' Organisations (HCPWP)

PCWP/HCPWP workshop on social media – 19 September 2016

PCWP/HCPWP joint meeting - 20 September 2016

Action: For information

Document(s) tabled:
Report of the PCWP/HCPWP workshop on social media – 19 Sep (EMA/625077/2016)
Minutes of the PCWP/HCPWP joint meeting - 20 Sept

7.4. Cooperation within the EU regulatory network

None

7.5. Cooperation with International Regulators

7.5.1. Food and Drug Administration (FDA)

Action: For information

Document tabled:
Draft Agenda December 13, 2016

7.5.2. Japanese Pharmaceuticals and Medical Devices Agency (PMDA)

None

7.5.3. The Therapeutic Goods Administration (TGA), Australia

None

7.5.4. Health Canada

None

7.6. Contacts of the COMP with external parties and interaction with the Interested Parties to the Committee

None

7.7. COMP work plan

7.7.1. COMP Work Plan 2017

Action: For information

Document(s) tabled:
COMP Work Plan 2017 adopted

7.8. Planning and reporting

7.8.1. List of all applications submitted/expected and the COMP coordinatorship distribution of valid applications submitted in 2017

Action: For information

7.8.2. Overview of orphan marketing authorisations/applications

Action: For information

8. Any other business

None

9. Explanatory notes

The notes below give a brief explanation of the main sections and headings in the COMP agenda and should be read in conjunction with the agenda or the minutes.

Abbreviations / Acronyms

CHMP: Committee for Medicinal Product for Human Use
COMP: Committee for Orphan Medicinal Products
EC: European Commission
OD: Orphan Designation
PA: Protocol Assistance
PDCO: Paediatric Committee
PRAC: Pharmacovigilance and Risk Assessment Committee
SA: Scientific Advice
SAWP: Scientific Advice Working Party

Orphan Designation (*section 2 Applications for orphan medicinal product designation*)

The orphan designation is the appellation given to certain medicinal products under development that are intended to diagnose, prevent or treat rare conditions when they meet a pre-defined set of criteria foreseen in the legislation. Medicinal products which get the orphan status benefit from several incentives (fee reductions for regulatory procedures (including protocol assistance), national incentives for research and development, 10-year market exclusivity) aiming at stimulating the development and availability of treatments for patients suffering from rare diseases.

Orphan Designations are granted by Decisions of the European Commission based on opinions from the COMP. Orphan designated medicinal products are entered in the Community Register of Orphan Medicinal Products.

Protocol Assistance (*section 3 Requests for protocol assistance with significant benefit question*)

The protocol assistance is the help provided by the Agency to the sponsor of an orphan medicinal product, on the conduct of the various tests and trials necessary to demonstrate the quality, safety and efficacy of the medicinal product in view of the submission of an application for marketing authorisation.

Sponsor

Any legal or physical person, established in the Community, seeking to obtain or having obtained the designation of a medicinal product as an orphan medicinal product.

Maintenance of Orphan Designation (*section 4 Review of orphan designation for orphan medicinal products for marketing authorisation*).

At the time of marketing authorisation, the COMP will check if all criteria for orphan designation are still met. The designated orphan medicinal product should be removed from the Community Register of Orphan Medicinal Products if it is established that the criteria laid down in the legislation are no longer met.

More detailed information on the above terms can be found on the EMA website: www.ema.europa.eu/