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SCIENCE MEDICINES HEALTH

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Inspections, Human Medicines Pharmacovigilance and Committees

Committee for Orphan Medicinal Products (COMP)

Draft agenda for the meeting on 10-11 April 2017

Chair: Bruno Sepodes – Vice-Chair: Lesley Greene

10 April 2017, 09:00-19:30, room 2F

11 April 2017, 08:30-19:30, 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 10-11 April 2017. See April 2017 COMP minutes (to be published post May 2017 COMP meeting).

1.2. Adoption of agenda

COMP agenda for 10-11 April 2017.

1.3. Adoption of the minutes

COMP minutes for 14-15 March 2017.

2. Applications for orphan medicinal product designation

2.1. For opinion

2.1.1. - EMA/OD/319/16

Treatment of acute myeloid leukaemia

Action: For adoption, Oral explanation to be held on 10 April 2017 at 09:30

Documents tabled:

Draft Summary report with response to LoQs

Notes:

There have been 49 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-β-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 dihydrochloride salt, EMEA/OD/028/09 Tosedostat, EMEA/OD/091/09 1-Cyclopropyl-3-[3-(5-morpholin-4-ylmethyl-1H-benzimidazol-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 pyrrolbenzodiazepine 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, EMA/OD/197/16 Ivosidenib

Designations withdrawn: EMEA/OD/065/02 2-chloro-9-[2-deoxy-2-fluoro-β-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 Ig-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, EMA/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

2.1.2. - EMA/OD/260/16

Treatment of glioma

Action: For adoption, Oral explanation to be held on 10 April 2017 at 15:30

Documents tabled:

Draft Summary report with response to LoQs

Notes:

There have been 43 designations for this condition: EMA/OD/026/03 Herpes simplex virus lacking infected cell protein 34.5, EMA/OD/055/03 Gimimatecan, EMA/OD/050/04 Biotinylated anti-tenascin monoclonal antibody for use with 90-Yttrium, EMA/OD/038/04 Anti epidermal growth factor receptor antibody h-R3, EMA/OD/030/05 Oligonucleotide phosphorothioate (TAAACGTTATAACGTTATGACGTCAT), sodium salt, EMA/OD/068/05 Enzastaurin hydrochloride, EMA/OD/110/05 4-[131I] iodo-L-phenylalanine, EMA/OD/081/06 Autologous dendritic cells pulsed with autologous tumour cell lysate, EMA/OD/050/07 Doxorubicin hydrochloride (drug eluting beads), EMA/OD/051/07 Irinotecan hydrochloride (drug eluting beads), EMA/OD/038/07 Iodine (131I) Chlorotoxin, EMA/OD/004/08 Recombinant fusion protein of circularly-permuted IL-4 and pseudomonas exotoxin A, [IL-4(38-37)-PE38KDEL], EMA/OD/023/08 Topotecan hydrochloride (liposomal), EMA/OD/034/08 Gadodiamide (liposomal), EMA/OD/104/08 Autologous tumour-derived gp96 heat shock protein-peptide complex, EMA/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 limonin, 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 Olaptosed pegol, EMA/OD/200/14 5,5'-(4-(trifluoromethyl) benzylazanediyloxy)bis(2,2,2-trifluoroethyl)phosphorothioate

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, EMA/OD/215/16 5-aminolevulinic acid

Designations withdrawn: EMEA/OD/004/02 Pseudomonas exotoxin (domains II/III)-Interleukin 13 chimeric protein, EMEA/OD/074/01 Human transferrin conjugated to mutant diphtheria 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] diazepam-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

2.1.3. - EMA/OD/323/16

Treatment of Herpes simplex encephalitis

Action: For adoption, Oral explanation to be held on 10 April 2017 at 17:00

Documents tabled:

Draft Summary report with response to LoQs

2.1.4. - EMA/OD/316/16

Treatment of Niemann-Pick disease, type C

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

Documents tabled:

Draft Summary report with response to LoQs

Notes:

There have been 5 designations for this condition: EMEA/OD/090/05 miglustat, EMA/OD/033/11 Hydroxy-propyl-beta-cyclodextrin, EMA/OD/160/12 Recombinant human heat shock protein 70, EMA/OD/191/12 2-hydroxypropyl-beta-cyclodextrin, EMA/OD/158/14 Arimocloamol citrate

2.2. For discussion / preparation for an opinion

2.2.1. - EMA/OD/017/17

Treatment of cystic fibrosis

Action: For adoption

Documents tabled:

Draft Summary report

Notes:

There have been 38 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-fluorophenyl)-[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, phosphatidylinositol 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'-trymethyangelicin, 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)-1H-indol-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/156/16 1-(2,2-difluoro-2H-1,3-benzodioxol-5-yl)-N-{1-[(2R)-2,3-dihydroxypropyl]-6-fluoro-2-(1-hydroxy-2-methylpropan-2-yl)-1H-indol-5-yl}cyclopropane-1-carboxamide and ivacaftor, 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

2.2.2. - EMA/OD/005/17

Treatment of glioma

Action: For adoption

Documents tabled:
Draft Summary report

Notes:

There have been 43 designations for this condition: Please see 2.1.2.

2.2.3. - EMA/OD/324/16

Treatment of spinal cord Injury

Action: For adoption

Documents tabled:
Draft Summary report

Notes:

There have been 4 designations for this condition: EMEA/OD/082/07 3-methoxy-pregnenolone, EMEA/OD/059/08 Recombinant human monoclonal antibody to human Nogo-A protein of the IgG4/kappa class, EMEA/OD/042/08 Filgrastim, EMA/OD/119/13 synthetic 12 amino acids peptide designed after subcommissural organ-spondin

Designation withdrawn: EMEA/OD/041/08 Autologous urothelial and smooth muscle cells

2.2.4. - EMA/OD/011/17

Treatment of cutaneous T-cell lymphoma

Action: For adoption

Documents tabled:
Draft Summary report

Notes:

There have been 14 designations for this condition: EMEA/OD/038/01 Denileukin diftitox, EMEA/OD/001/04 Human monoclonal antibody against CD4, EMEA/OD/001/05 (E)-(1S,4S,10S,21R)-7-[(Z)-ethylidene]-4,21-diisopropyl-2-oxa-12,13-dithia-5,8,20,23-tetraazabicyclo[8.7.6]tricos-16-ene-3,6,9,19,22-pentone, EMEA/OD/030/08 Miltefosine, EMEA/OD/135/09 Pralatrexate, EMA/OD/112/11 Chlormethine, EMA/OD/100/11 Brentuximab vedotin, EMA/OD/050/12 Naloxone hydrochloride dihydrate, EMA/OD/066/12 Recombinant anti-CD3-bi-single-chain-Fv-diphtheria toxin fusion protein, EMA/OD/084/14 Humanised IgG1 monoclonal antibody against human KIR3DL2, EMA/OD/033/15 Synthetic hypericin, EMA/OD/254/15 Resiquimod, EMA/OD/203/15 Fenretinide, EMA/OD/091/16 Mogamulizumab

Designations withdrawn: EMEA/OD/007/03 Adenovirus-Interferon gamma-coding DNA sequence, EMEA/OD/003/04 Suberolylanilide Hydroxamic acid, EMEA/OD/015/07 Panobinostat lactate

2.2.5. - EMA/OD/008/17

Treatment of sickle cell disease

Action: For adoption

Documents tabled:
Draft Summary report

Notes:

There have been 12 designations for this condition: EMA/OD/017/05 Extract of Sorghum bicolor leaf, Pterocarpus osun stem, Piper guineense seed and Caryophylli flower, EMA/OD/107/08 2,2-dimethylbutyric acid, sodium salt, EMA/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-oroethylamido-cyclohexane-1-carboxylic acid (ethyl-2-amidyl-ethoxy-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

2.2.6. - EMA/OD/248/16

Prevention of arteriovenous access dysfunction in haemodialysis patients

Action: For adoption

Documents tabled:
Draft Summary report

Notes:

There have been 1 designation for this condition: EMA/OD/139/13 Recombinant human type I pancreatic elastase

2.2.7. - EMA/OD/013/17

Treatment of growth hormone deficiency

Action: For adoption

Documents tabled:
Draft Summary report

Notes:

There have been 2 designations for this condition: EMA/OD/133/12 Recombinant modified human growth hormone, EMA/OD/074/13 Recombinant human growth hormone modified by fusion with two hydrophilic polypeptide chains

2.2.8. - EMA/OD/311/16

Treatment of neonatal abstinence syndrome

Action: For adoption

Documents tabled:

Draft Summary report

2.2.9. - EMA/OD/004/17

Treatment of paroxysmal nocturnal haemoglobinuria

Action: For adoption

Documents tabled:

Draft Summary report

Notes:

There have been 5 designations for this condition: EMEA/OD/042/03 Eculizumab, EMA/OD/098/14 S3,S13-cyclo(D-tyrosyl-L-isoleucyl-L-cysteinyl-L-valyl-1-methyl-L-tryptophyl-L-glutamyl-L-aspartyl-L-tryptophyl-N-methyl-L-glycyl-L-alanyl-L-histidyl-L-arginyl-L-cysteinyl-N-methyl-L-isoleucinamide), EMA/OD/246/15 Fc- and CDR-modified humanised monoclonal antibody against C5, EMA/OD/107/16 Synthetic-15-amino acid macrocyclic peptide acylated with a polyethyleneglycol palmitoylated linker, EMA/OD/077/16 Recombinant protein derived from the saliva of the *Ornithodoros moubata* tick

Designation withdrawn: EMEA/OD/016/02 Myristolated-peptidyl-recombinant Human CD59

2.2.10. - EMA/OD/019/17

Treatment of haemophilia A

Action: For adoption

Documents tabled:

Draft Summary report

Notes:

There have been 11 designations for this condition: EMA/OD/128/10 Pegylated B-domain-deleted sequence-modified recombinant human factor VIII, EMA/OD/132/10 Recombinant fusion protein linking human coagulation factor VIIa with human albumin (rVIIa-FP), EMA/OD/144/11 Pegylated recombinant factor VIII, EMA/OD/095/12 Humanised monoclonal IgG4 antibody against tissue factor pathway inhibitor, EMA/OD/039/14 Synthetic double-stranded siRNA oligonucleotide directed against antithrombin mRNA that is covalently linked to a ligand containing three N-acetylgalactosamine residues, EMA/OD/144/13 Humanised monoclonal modified IgG4 antibody with bispecific structure targeting factors IX, IXa, X and Xa, EMA/OD/069/14 Recombinant factor VIIa modified with three terminal repeats derived from the β chain of human chorionic gonadotropin, EMA/OD/123/14 A combination of H-Lys-Lys-Gly-Pro-Arg-Cys(SH)-Leu-Thr-Arg-Tyr-Tyr-Ser-Ser-Phe-Val-Asn-Met-Glu-Gly-Lys-Lys-OH and H-Lys-Lys-Gly-Asp-Asn-Ile-Met-Val-Thr-Phe-Arg-Asn-Gln-Ala-Ser-Arg-Pro-Tyr-Gly-Lys-Lys-OH, EMA/OD/238/16 Autologous dendritic cells incubated ex vivo with zebularine and factor VIII, EMA/OD/230/15 adeno-associated viral vector serotype 5 containing a B-domain deleted variant of human coagulation factor VIII gene, EMA/OD/093/16 Human monoclonal IgG1 antibody against tissue factor pathway inhibitor

Designations withdrawn: EMEA/OD/031/09 Sequence-modified recombinant human factor VIIa, EMA/OD/030/10 Recombinant fusion protein consisting of human coagulation factor

VIII attached to the Fc domain of human IgG1, EMA/OD/043/10 Recombinant porcine factor VIII (B domain deleted), EMA/OD/069/12 vatreptacog alfa (activated)

2.2.11. - EMA/OD/018/17

Treatment of haemophilia B

Action: For adoption

Documents tabled:

Draft Summary report

Notes:

There have been 8 designations for this condition: EMEA/OD/005/09 Pegylated recombinant human factor IX, EMEA/OD/117/09 Recombinant fusion protein linking human coagulation factor IX with human albumin, EMA/OD/133/10 Recombinant fusion protein linking human coagulation factor VIIa with human albumin, EMA/OD/090/11 Adeno-associated viral vector containing the human factor IX gene, EMA/OD/041/14 Synthetic double-stranded siRNA oligonucleotide directed against antithrombin mRNA that is covalently linked to a ligand containing three N-acetylgalactosamine residues, EMA/OD/073/14 Recombinant factor VIIa modified with three terminal repeats derived from the β chain of human chorionic gonadotropin, EMA/OD/003/15 Adeno-associated viral vector containing the human factor IX gene, EMA/OD/172/15 Adeno-associated virus viral vector serotype rh10 encoding containing the human factor IX gene

Designations withdrawn: EMEA/OD/008/08 Pegylated recombinant factor VIIa, EMEA/OD/062/09 Sequence modified human recombinant factor VIIa, EMA/OD/070/12 vatreptacog alfa (activated)

2.2.12. - EMA/OD/321/16

Treatment of idiopathic CD4 lymphocytopenia

Action: For adoption

Documents tabled:

Draft Summary report

2.2.13. - EMA/OD/014/17

Treatment of ovarian cancer

Action: For adoption

Documents tabled:

Draft Summary report

Notes:

There have been 30 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/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 Vincalukoblastin-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, EMA/OD/114/12 Alisertib

2.2.14. - EMA/OD/304/16

Prevention of arterial pulmonary hypertension

Action: For adoption

Documents tabled:

Draft Summary report

2.2.15. - EMA/OD/001/17

Prevention of rejection following solid organ transplantation

Action: For adoption

Documents tabled:

Draft Summary report

2.2.16. - EMA/OD/007/17

Treatment of tuberous sclerosis

Action: For adoption

Documents tabled:

Draft Summary report

Notes:

There have been 2 designations for this condition: EMA/OD/010/10 Everolimus, EMA/OD/100/15 Sirolimus

2.2.17. - EMA/OD/010/17

Treatment of acute myeloid leukaemia

Action: For adoption

Documents tabled:

Draft Summary report

Notes:

There have been 49 designations for this condition: Please see 2.1.1.

2.2.18. - EMA/OD/020/17

Treatment of subarachnoid haemorrhage (SAH)

Action: For adoption

Documents tabled:

Draft Summary report

2.2.19. - EMA/OD/002/17

Treatment of congenital hyperinsulinism

Action: For adoption

Documents tabled:

Draft Summary report

Notes:

There have been 3 designations for this condition: EMA/OD/140/11 Glucagon, EMA/OD/128/14 Glucagon, EMA/OD/040/16 Recombinant human monoclonal antibody to insulin receptor

2.2.20. - EMA/OD/006/17

Treatment of cystic fibrosis

Action: For adoption

Documents tabled:

Draft Summary report

Notes:

There have been 38 designations for this condition: Please see 2.2.1.

2.2.21. - EMA/OD/016/17

Treatment of distal renal tubular acidosis

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. Ciclosporin – EMA/OD/022/04, EU/3/04/210

PARI Pharma GmbH - Germany; Treatment of graft rejection after lung transplantation;

Action: For discussion

Documents 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 10-11 April 2017 COMP meeting

2.7. Evaluation on-going

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

Action: For information

Notes:

Cross reference to other agenda point. See 7.8.1. Table 6. Evaluation Ongoing.

3. Requests for protocol assistance with significant benefit question

3.1. Ongoing procedures

3.1.1. -

Treatment of Wolfram syndrome

Action: For discussion

3.1.2. -

Treatment of Wolfram syndrome

Action: For discussion

3.1.3. -

Treatment of soft tissue sarcoma

Action: For adoption

3.1.4. -

Treatment of haemophilia A

Action: For adoption

3.1.5. -

Treatment of beta-thalassemia intermedia and major

Action: For adoption

3.2. Finalised letters

3.2.1. -

Treatment of Gaucher disease

Action: For information

3.2.2. -

Treatment of narcolepsy

Action: For information

3.2.3. -

Treatment of Langerhans cell histiocytosis

Action: For information

3.2.4. -

Treatment of paroxysmal nocturnal haemoglobinuria

Action: For information

3.3. New requests

3.3.1. -

Treatment of myasthenia gravis

Action: For information

3.3.2. -

Prevention of graft-versus-host disease

Action: For information

3.3.3. -

Treatment of mercury toxicity

Action: For information

4. Review of orphan designation for orphan medicinal products for marketing authorisation

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

4.1.1. Refixia - nonacog beta pegol – EMEA/OD/005/09, EU/3/09/640, EMEA/H/C/004178

Novo Nordisk A/S; Treatment of haemophilia B

Action: For adoption, Oral explanation to be held on 10 April 2017 at 11:00

Documents tabled:

Draft report on review of OMPD

Notes:

Status of the procedure at the CHMP: Opinion adopted in March 2017

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

Bene-Arzneimittel GmbH; Treatment of interstitial cystitis

Action: For adoption, Oral explanation to be held on 10 April 2017 at 12:00

Documents tabled:

Draft report on review of OMPD

Notes:

Status of the procedure at the CHMP: Opinion adopted in March 2017

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

4.2.1. - cenegermin - EMEA/H/C/004209, EMA/OD/143/15, EU/3/15/1586

Dompe farmaceutici s.p.a.; Treatment of neurotrophic keratitis

Action: For discussion

Document(s) tabled:

Draft report on review of OMPD

4.2.2. - trientine tetrahydrochloride – EMEA/H/C/004005/000, EMA/OD/001/15, EU/3/15/1471

GMP-Orphan SA; Treatment of Wilson's disease

Action: For information

Document(s) tabled:

Draft report on review of OMPD

4.2.3. - inotuzumab ozogamicin – EMEA/H/C/004119, EMA/OD/194/12, EU/3/13/1127

Pfizer Limited; Treatment of B-cell acute lymphoblastic leukaemia

Action: For discussion

Document(s) tabled:

Draft report on review of OMPD

4.2.4. - masitinib – EMEA/OD/062/04, EMEA/H/C/004159, EU/3/04/242

AB Science; Treatment of Mastocytosis

Action: For information

Document(s) tabled:

Draft report on review of OMPD

4.2.5. - cerliponase alfa - EMA/OD/177/12, EU/3/13/1118, EMEA/H/C/004065

BioMarin International Limited; Treatment of neuronal ceroid lipofuscinosis type 2

Action: For discussion

Documents tabled:

Draft report on review of OMPD

4.3. Appeal

None

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

None

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

None

7. Organisational, regulatory and methodological matters Mandate and organisation of the COMP

7.1. Mandate and organisation of the COMP

7.1.1. Strategic Review & Learning meetings

None

7.1.2. Protocol Assistance Working Group

Proposed meeting time on 11 April 2017 at 12:00

Document(s) tabled:

PAWG draft agenda for April 2017 meeting

PAWG draft minutes for March 2017 meeting

7.1.3. Preclinical Models Working Group

Proposed meeting time on 11 April 2017 at 08:00

7.1.4. Conditions Steering Group

Proposed meeting time on 11 April 2017 at 18:30

7.2. Coordination with EMA Scientific Committees or CMDh-v

7.2.1. PDCO/COMP Working Group

Proposed meeting time on 11 April 2017 at 13:00 (TBC)

7.2.2. Recommendations on eligibility to PRIME – report from CHMP

Action: For information

Document(s) tabled:

PRIME eligibility requests - list of adopted outcomes March 2017

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

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

PCWP meeting with all eligible organisations – 30 November 2016

Action: For information

Document(s) tabled:

Minutes of the PCWP meeting with all eligible organisations – 30 Nov (EMA/801985/2016)

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

PCWP/HCPWP workshop on personalised medicines: role of patients, consumers and healthcare professionals – 14 March 2017

PCWP/HCPWP joint meeting - 15 March 2017

Action: For information

Document(s) tabled:

Agenda of the Workshop on personalised medicines: role of patients, consumers and healthcare professionals - 14 March 2017 (EMA/762357/2016)

Agenda of the PCWP/HCPWP joint meeting – 15 March 2017 (EMA/69326/2017)

7.4. Cooperation within the EU regulatory network

7.4.1. European Commission

None

7.5. Cooperation with International Regulators

7.5.1. Food and Drug Administration (FDA)

None

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

7.6.1. EMA framework of collaboration with academia

Action: For information

Document(s) tabled:
Presentation

7.7. COMP work plan

7.7.1. COMP Work Plan 2017

Action: For information

Document(s) tabled:
COMP Work Plan 2017

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/