6 September 2016
EMA/COMP/502043/2016
Procedure Management and Committees Support Division

Committee for Orphan Medicinal Products (COMP)

Draft agenda for the meeting on 6-8 September 2016

Chair: Bruno Sepodes – Vice-Chair: Lesley Greene

06 September 2016, 09:00-19:30, room 2F

07 September 2016, 08:30-19:30, room 2F

08 September 2016, 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 6-8 September 2016. See September 2016 COMP minutes (to be published post October 2016 COMP meeting).

1.2. Adoption of agenda

COMP agenda for 6-8 September 2016.

1.3. Adoption of the minutes

COMP minutes for 11-13 July 2016.

2. Applications for orphan medicinal product designation

2.1. For opinion

2.1.1. - EMA/OD/089/16

Treatment of gastroenteropancreatic neuroendocrine tumours

Action: For adoption, Oral explanation to be held on 6 September 2016 at time 09:30

Documents tabled:

Draft Summary report with response to LoQs

Notes:
There have been 4 designations for this condition: EMA/OD/173/13 68Ga-2,2'-(7-(4-((S)-1-((4S,7S,10S,13R,16S,19R)-4-((R)-1-amino-3-(4-hydroxyphenyl)-1-oxopropan-2-ylcarbamoyl)-10-(4-aminobutyl)-16-(4-((S)-2,6-dioxohexahydropyrimidine-4-carboxamido)benzyl)-7-((R)-1-hydroxyethyl)-6,9,12,15,18-pentaoxo-13-(4-ureidobenzyl)-1,2-dithia-5,8,11,14,17-pentaazacycloicosan-19-ylamino)-3-(4-chlorophenyl)-1-oxopropan-2-ylamino)-1-carboxy-4-oxobutyl)-1,4,7-triazonane-1,4-diyl)diacetic acid, EMA/OD/219/14 Gallium (68Ga)-edotreotide, EMEA/OD/005/04 (2-aminoethyl) carbamic acid (2R,5S,8S,11S,14R,17S,19aS)-11-(4-aminobutyl)-5-benzyl-8-(4-benzyloxy benzyl)-14-(1H-indol-3-ylmethyl)-4,7,10,13,16,19-hexaoxo-17-phenyloctadecahydro-3a,6,9,12,15,18-hexaazacyclopentacyclooctadecen-2-yl ester, di[(S)-2-aminosuccinic acid] salt, EMA/OD/211/15 Fosbretabulin tromethamine

2.1.2. - EMA/OD/099/16

Treatment of periventricular leukomalacia

Action: For adoption, Oral explanation to be held on 6 September 2016 at time 14:00

Documents tabled:
Draft Summary report with response to LoQs

2.1.3. - EMA/OD/115/16

Treatment of myelofibrosis

Action: For adoption, Oral explanation to be held on 6 September 2016 at time 15:00

Documents tabled:
Draft Summary report with response to LoQs

Notes:
There has been 1 designation for this condition: EMA/OD/154/15 Imetelstat sodium

2.1.4. - EMA/OD/113/16

Treatment of retinitis pigmentosa

Action: For adoption, Oral explanation to be held on 6 September 2016 at time 16:30

Documents tabled:
Draft Summary report with response to LoQs

Notes:
There have been 16 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/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

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

2.1.5. - EMA/OD/084/16

Treatment of short bowel syndrome

Action: For information

Documents tabled:
Withdrawal request of 9 August 2016

2.1.6. - EMA/OD/091/16

Treatment of cutaneous T-cell lymphoma

Action: For adoption, Oral explanation to be held on 6 September 2016 at time 18:30

Documents tabled:
Draft Summary report with response to LoQs

Notes:
There have been 13 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

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

2.1.7. - EMA/OD/100/16

Treatment of cystic fibrosis

Action: For adoption, Oral explanation to be held on 7 September 2016 at time 09:00

Documents tabled:
Draft Summary report with response to LoQs

Notes:
There have been 37 designations for this condition: EMEA/OD/011/03 Recombinant dog gastric lipase , EMEA/OD/032/00 L-Lysine-N-Acetyl-L-Cysteinate, 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/072/05 Denufosol tetrasodium , 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

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/118/05 Glutathione , EMEA/OD/024/08 Levofloxacin hemihydrate , EMA/OD/032/14 Lumacaftor/ivacaftor

2.1.8. - EMA/OD/202/15

Treatment of variegate porphyria

Action: For adoption, Oral explanation to be held on 7 September 2016 at time 10:00

Documents tabled:
Draft Summary report with response to LoQs

2.1.9. - EMA/OD/112/16

Treatment of narcolepsy

Action: For adoption, Oral explanation to be held on 7 September 2016 at time 11:00

Documents tabled:
Draft Summary report with response to LoQs

Notes:

There have been 3 designations for this condition: EMEA/OD/087/06 1-{3-[3-(4-chlorophenyl)propoxy]propyl}piperidine, hydrochloride , EMA/OD/254/14 Mazindol , EMA/OD/002/15 Mazindol

Designation withdrawn: EMEA/OD/051/02 Sodium oxybate

2.1.10. - EMA/OD/108/16

Treatment of soft tissue sarcoma

Action: For adoption, Oral explanation to be held on 7 September 2016 at time 12:00

Documents tabled:
Draft Summary report with response to LoQs

Notes:
There have been 10 designations for this condition: EMEA/OD/001/01 Ecteinascidin 743, EMEA/OD/042/06 Doxorubicin hydrochloride (liposomal), EMA/OD/155/11 Yttrium (90Y)-DTPA-radiolabelled chimeric monoclonal antibody against frizzled homologue 10 , EMA/OD/041/13 Genetically modified serotype 5/3 adenovirus coding for granulocyte macrophage colony-stimulating factor , EMA/OD/190/13 Doxorubicin(6-maleimidocaproyl)hydrazone , EMA/OD/266/14 Olaratumab , EMA/OD/159/15 Glucopyranosyl lipid A stable emulsion and recombinant New York esophageal squamous cell carcinoma-1 protein , EMA/OD/184/15 (S)-N-(5-((R)-2-(2,5-difluorophenyl)pyrrolidin-1-yl)pyrazolo[1,5-a]pyrimidin-3-yl)-3-hydroxypyrrolidine-1-carboxamide hydrogen sulfate , EMA/OD/215/15 Human/murine chimeric monoclonal antibody against endoglin , EMA/OD/238/15 Sindbis virus envelope pseudotyped lentiviral vector encoding New York esophageal squamous cell carcinoma-1 protein

Designations withdrawn: EMEA/OD/059/03 N-acetylsarcosyl-glycyl-L-valyl-D-alloisoleucyl-L-threonyl-L-norvalyl-L-isoleucyl-L-arginyl-L-prolyl-N-ethylamide , EMEA/OD/050/05 27R,34aS)-9,27-dihydroxy-10,21-dimethoxy-6,8,12,14,20,26-hexamethyl-1,5,11,28,29-pentaoxo-1,4,5,6,9,10,11,12,13,14,21,22,23,24,25,26,27,28,29,31,32,33,34,34a-tetra-cosahydro-3H-23,27-epoxypyrido[2,1-c][1,4]oxazacyclohentriacontin-3-yl]propyl}-2-methoxy-cyclohexyldimethyl-phosphinate, EMEA/OD/071/05 Brostallicin, EMEA/OD/083/06 Fenretinide, EMEA/OD/044/08 Palifosfamide, EMA/OD/141/10 Ombrabulin, EMA/OD/110/11 (1-methyl-2-nitro-1H-imidazole-5-yl)methyl N,N'-bis(2-bromoethyl) diamidophosphate

2.1.11. - EMA/OD/105/16

Treatment of progressive multifocal leukoencephalopathy

Action: For information

Documents tabled:
Withdrawal request of 11 August 2016

2.1.12. - EMA/OD/090/16

Treatment of acute myeloid leukaemia

Action: For adoption, Oral explanation to be held on 7 September 2016 at time 16:30

Documents tabled:

Draft Summary report with response to LoQs

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/156/10 Allogeneic umbilical cord blood 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-Ph…..., 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

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/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

2.1.13. - EMA/OD/109/16

Treatment of global ischaemic reperfusion injury

Action: For adoption, Oral explanation to be held on 7 September 2016 at time 17:30

Documents tabled:
Draft Summary report with response to LoQs

2.1.14. - EMA/OD/126/16

Treatment of Duchenne muscular dystrophy

Action: For adoption, Oral explanation to be held on 7 September 2016 at time 18:30

Documents tabled:

Draft Summary report with response to LoQs

Notes:
There have been 24 designations for this condition: EMEA/OD/106/04 3-[5-(2-fluoro-phenyl)-[1,2,4]oxadiazole-3-yl]-benzoic acid, EMEA/OD/026/05 Adeno-associated viral vector containing a modified U7 snRNA gene, EMEA/OD/077/06 Idebenone, EMEA/OD/065/08 5-(ethylsulfonyl)-2-(naphthalen-2-yl)benzo[d]oxazole, EMEA/OD/049/08 RNA, [P-deoxy-P-(dimethylamino)] (2',3'-dideoxy-2',3'-imino-2',3'-seco) (2'a→5') (C-m5U-C-C-A-A-C-A-m5U-C-A-A-G-G-A-A-G-A-m5U-G-G-C-A-m5U-m5U-m5U-C-m5U-A-G), P-[4-[[2-[2-(2-hydroxyethoxy)ethoxy]ethoxy]carbonyl]-1-piperazinyl] N,N-dimethylam, EMEA/OD/081/08 Exon 44 specific phosphorothioate oligonucleotide, EMEA/OD/082/08 Exon 51 specific phosphorothioate oligonucleotide, EMEA/OD/044/09 Adeno-associated viral

vector containing modified U1 snRNA, EMEA/OD/083/09 RNA, [P-deoxy-P-(dimethylamino)] (2',3'-dideoxy-2',3'-imino-2',3'-seco) (2'a→5') (C-m5U-m5U-A-C-A-G-G-C-m5U-C-C-A-A-m5U-A-G-m5U-G-G-m5U-C-A-G-m5U), 5' [P-[4-[[2-[2-(2-hydroxyethoxy)ethoxy]ethoxy]carbonyl]-1-piperazinyl]-N,N-dimethylaminophosphonamidate], 3'-[2'a-[N2-acetyl-L-arginyl-6-aminohexanoyl-L-arginyl-L-arginyl-β-alanyl-L-arginyl-L-arginyl-6-aminohexanoyl-L-arginyl-L-arginyl-β-alanyl-L-arginyl-6-aminohexanoyl-β-alanyl], octahydrochloride, EMA/OD/090/13 Naproxcinod, EMA/OD/142/11 Exon 45 specific phosphorothioate oligonucleotide, EMA/OD/143/11 Exon 53 specific phosphorothioate oligonucleotide , EMA/OD/162/11 Halofuginone hydrobromide , EMA/OD/028/12 Givinostat, EMA/OD/121/12 Exon 52 specific phosphorothioate oligonucleotide , EMA/OD/122/12 Exon 55 specific phosphorothioate oligonucleotide , EMA/OD/164/12 Humanised monoclonal antibody against myostatin , EMA/OD/183/12 R,S-O-(3-piperidino-2-hydroxy-1-propyl)-nicotinic acid amidoxime dihydrochloride , EMA/OD/162/13 Asp-Arg-Val-Tyr-Ile-His-Pro , EMA/OD/049/14 17α,21-dihydroxy-16α-methyl-pregna-1,4,9(11)-triene-3,20-dione , EMA/OD/166/14 Adeno-associated viral vector serotype 8 containing the human MD1 gene , EMA/OD/307/14 Rimeporide, EMA/OD/041/15 Allogeneic human adult stem cells, isolated from skeletal muscle and expanded ex vivo , EMA/OD/109/15 N-(2-((4Z,7Z,10Z,13Z,16Z,19Z)-docosa-4,7,10,13,16,19-hexaenamido)ethyl)-2-hydroxybenzamide

Designations withdrawn: EMEA/OD/096/05 2'-O-methyl-phosphorothioate oligonucleotide, EMEA/OD/025/06 2-(4-(diethylamino) phenyl)-6-methyl-2H-benzo[d][1,2,3] triazol-5-amine, EMA/OD/085/10 Recombinant fusion protein consisting of the extracellular portion of human activin receptor IIB linked to the human IgG1 Fc domain

2.1.15. - EMA/OD/123/16

Treatment of osteomyelitis

Action: For adoption, Oral explanation to be held on 8 September 2016 at time 09:00

Documents tabled:
Draft Summary report with response to LoQs

2.1.16. - EMA/OD/121/16

Treatment of multiple myeloma

Action: For adoption, Oral explanation to be held on 8 September 2016 at time 11:00

Documents tabled:
Draft Summary report with response to LoQs

Notes:
There have been 13 designations for this condition: EMEA/OD/040/01 Thalidomide, EMEA/OD/063/03 3-(4'aminoisoindoline-1'-one)-1-piperidine-2,6-dione, EMEA/OD/044/04 Aplidine , EMEA/OD/066/04 Recombinant histidine-tagged idiotype immunoglobulin Fab fragment of clonal B-cell receptors , EMEA/OD/012/05 N-(methyl-diazacyclohexyl-methylbenzamide)-azaphenyl-aminothiopyrrole , EMEA/OD/120/07 Carfilzomib , EMEA/OD/068/08 N2'-Deacetyl-N2'-[4-methyl-4-(oxobuthyldithio)-1-oxopentyl]-maytansine-chimerized anti-CD138 IgG4 monoclonal antibody , EMEA/OD/076/08 Human anti-intercellular adhesion molecule-1 monoclonal antibody , EMEA/OD/053/08 Milatuzumab

, EMEA/OD/053/09 Pomalidomide , EMA/OD/017/11 Acadesine , EMA/OD/048/11 2,2'-{2-[(1R)-1-({[(2,5-dichlorobenzoyl)amino]acetyl}amino)-3-methylbutyl]-5-oxo-1,3,2-dioxaborolane-4,4-diyl}diacetic acid, EMA/OD/113/12 Panobinostat

Designations withdrawn: EMEA/OD/048/00 Arsenic trioxide, EMEA/OD/003/01 Humanised anti-HM1.24 monoclonal antibody, EMEA/OD/018/00 Thalidomide, EMEA/OD/026/01 Deoxyribose phosphorothioate (5'-tct-ccc-agc-gtg-cgc-cat-3'), EMEA/OD/019/01 Thalidomide, EMEA/OD/070/04 17-allylamino-17-demethoxygeldanamycin , EMEA/OD/093/05 Human monoclonal antibody against HLA-DR , EMEA/OD/003/09 Chimeric-anti-interleukin-6 monoclonal antibody , EMEA/OD/133/09 Dexamethasone (40 mg tablet) , EMEA/OD/130/09 Perifosine , EMA/OD/115/10 Maytansinoid-conjugated humanised monoclonal antibody against CD56 , EMA/OD/137/10 Vorinostat , EMA/OD/137/11 Chimeric monoclonal antibody against kappa myeloma antigen, EMA/OD/061/12 Elotuzumab

2.1.17. - EMA/OD/107/16

Treatment of paroxysmal nocturnal haemoglobinuria

Action: For adoption, Oral explanation to be held on 8 September 2016 at time 12:00

Documents tabled:
Draft Summary report with response to LoQs

Notes:
There have been 3 designations for this condition: EMEA/OD/042/03 Eculizumab , EMA/OD/098/14 S3,S13-cyclo(D-tyrolsyl-L-isoleucyl-L-cysteinyl-L-valyl-1-methyl-L-tryptophyl-L-glutaminyl-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

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

2.1.18. - EMA/OD/092/16

Treatment of haemophilia B

Action: For adoption, Oral explanation to be held on 8 September 2016 at time 14:00

Documents tabled:
Draft Summary report with response to LoQs

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)

2.1.19. - EMA/OD/093/16

Treatment of haemophilia A

Action: For adoption, Oral explanation to be held on 8 September 2016 at time 14:00

Documents tabled:
Draft Summary report with response to LoQs

Notes:
There have been 9 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/230/15 adeno-associated viral vector serotype 5 containing a B-domain deleted variant of human coagulation factor VIII gene

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)

2.1.20. - EMA/OD/104/16

Treatment of invasive aspergillosis

Action: For adoption

Documents tabled:
Draft Summary report with response to LoQs

Notes:
There has been 1 designation for this condition: EMA/OD/009/14 Isavuconazonium sulfate

2.1.21. - EMA/OD/087/16

Treatment of diffuse large B cell lymphoma

Action: For adoption

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

2.1.22. - EMA/OD/122/16

Treatment of diffuse large B-cell lymphoma

Action: For adoption

Documents tabled:
Draft Summary report with response to LoQs

Notes:
There have been 8 designations for this condition: EMEA/OD/091/08 Recombinant hisitidine-tagged idiotype immunoglobulin Fab fragment of clonal B-cell receptors , EMA/OD/160/10 Lenalidomide , EMA/OD/116/13 Ibrutinib , EMA/OD/092/14 obinutuzumab, EMA/OD/215/14 Humanised Fc engineered monoclonal antibody against CD19, EMA/OD/005/15 Humanised anti-CD37 monoclonal antibody conjugated to maytansinoid DM1, EMA/OD/016/16 3-(5-amino-2-methyl-4-oxoquinazolin-3(4H)-yl)piperidine-2,6-dione hydrochloride, EMA/OD/084/15 2'-deoxyguanosylyl-(3',5'-phosphoryl)-2'-deoxythymidylyl-(3',5'-phosphoryl)- 2'-deoxyguanosylyl-(3',5'-phosphoryl)-2'-deoxycytidylyl-(3',5'-phosphoryl)-2'-deoxycytidylyl -(3',5'-phosphoryl)-2'-deoxycytidylyl-(3',5'-phosphoryl)-2'-deoxyguanos

Designation withdrawn: EMEA/OD/126/09 Pixantrone dimaleate

2.2. For discussion / preparation for an opinion

2.2.1. - EMA/OD/132/16

Treatment of X-linked adrenoleukodystrophy

Action: For adoption

Documents tabled:
Draft Summary report

2.2.2. - EMA/OD/128/16

Treatment of Smith-Magenis syndrome

Action: For adoption

Documents tabled:
Draft Summary report

2.2.3. - EMA/OD/146/16

Treatment of retinitis punctata albescens

Action: For adoption

Documents tabled:
Draft Summary report

2.2.4. - EMA/OD/133/16

Treatment of Crigler-Najjar syndrome

Action: For adoption

Documents tabled:
Draft Summary report

Notes:
There have been 4 designations for this condition: EMA/OD/082/14 Adeno-associated viral vector serotype 8 containing the human UGT1A1 gene, EMA/OD/122/14 Adeno-associated viral vector serotype 8 containing the human UGT1A1 gene, EMEA/OD/039/07 Heterologous human adult liver derived stem cells, EMA/OD/047/16 Modified mRNA encoding the UGT1A1 protein

2.2.5. - EMA/OD/158/16

Treatment of retinitis pigmentosa

Action: For adoption

Documents tabled:
Draft Summary report

Notes:
There have been 16 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/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

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/151/16

Treatment of cytomegalovirus infection in patients with impaired cell-mediated immunity

Action: For adoption

Documents tabled:
Draft Summary report

Notes:
There have been 3 designations for this condition: EMA/OD/008/12 Letermovir, EMA/OD/246/14 Allogeneic CD4+ and CD8+ T lymphocytes ex vivo incubated with synthetic peptides of the viral antigens of cytomegalovirus, adenovirus and Epstein-Barr virus, EMA/OD/234/15 Brincidofovir

2.2.7. - EMA/OD/149/16

Prevention of graft-versus-host disease

Action: For adoption

Documents tabled:
Draft Summary report

Notes:
There have been 7 designations for this condition: EMEA/OD/054/06 Recombinant fusion protein consisting of the extracellular portion of CD95 fused to the Fc part of a human IgG1 molecule , EMEA/OD/121/07 Donor lymphocyte preparation depleted of functional alloreactive T-cells , EMA/OD/103/13 Defibrotide , EMA/OD/146/13 Allogeneic bone-marrow derived ex-vivo expanded multipotent adult progenitor cells, EMA/OD/163/14 Allogeneic bone marrow derived mesenchymal cells expanded ex vivo in synthetic media, EMA/OD/119/15 Humanised fusion protein consisting of extracellular domain of CD24 linked to IgG1 Fc domain, EMA/OD/131/15 2-amino-2-[2-[2-chloro-4-[[3-(phenylmethoxy)phenyl]thio]phenyl]ethyl]-1,3-propanediol hydrochloride

2.2.8. - EMA/OD/167/15

Treatment of acquired Factor Xa coagulopathy associated with severe, life threatening bleeding in a critical organ or compartment

Action: For adoption

Documents tabled:
Draft Summary report

2.2.9. - EMA/OD/141/16

Treatment of hypoxic-ischaemic encephalopathy

Action: For adoption

Documents tabled:
Draft Summary report

2.2.10. - EMA/OD/139/16

Treatment of IgA nephropathy

Action: For adoption

Documents tabled:
Draft Summary report

2.2.11. - EMA/OD/148/16

Treatment of metaphyseal chondrodysplasia, Schmid type

Action: For adoption

Documents tabled:
Draft Summary report

2.2.12. - EMA/OD/142/16

Treatment of mucopolysaccharidosis type IIIA (Sanfilippo A syndrome)

Action: For adoption

Documents tabled:
Draft Summary report

Notes:
There have been 5 designations for this condition: EMA/OD/042/10 adenovirus-associated viral vector serotype 10 carrying the human N-sulfoglucosamine sulfohydrolase and sulfatase modifying factor 1 cDNAs , EMA/OD/171/10 Adeno-associated viral vector serotype 9 containing the human sulfamidase gene , EMA/OD/006/14 Autologous CD34+ cells transduced with a lentiviral vector containing the human SGSH gene, EMA/OD/164/14 Adeno-associated viral vector serotype rh.10 carrying the human N-sulfoglucosamine sulfohydrolase cDNA, EMEA/OD/052/08 Recombinant human heparan-N-sulfatase

2.2.13. - EMA/OD/103/16

Treatment of ovarian cancer

Action: For adoption

Documents tabled:
Draft Summary report

Notes:

There have been 31 designations for this condition: EMEA/OD/019/02 Oregovomab, EMEA/OD/080/03 Anti-epithelial cell adhesion molecule/anti-CD3 monoclonal antibody , EMEA/OD/044/03 Trabectedin , EMEA/OD/061/06 Paclitaxel (micellar) , 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, 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/094/11 Vincaleukoblastin-23-oic acid, O4-deacetyl-2-[(2-mercaptoethoxy)carbonyl]hydrazide, disulfide with …, 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/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

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/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

2.2.14. - EMA/OD/129/16

Treatment of soft tissue sarcoma

Action: For adoption

Documents tabled:
Draft Summary report

Notes:

There have been 12 designations for this condition: EMEA/OD/001/01 Ecteinascidin 743, EMEA/OD/042/06 Doxorubicin hydrochloride (liposomal) , EMA/OD/155/11 Yttrium (90Y)-DTPA-radiolabelled chimeric monoclonal antibody against frizzled homologue 10, EMA/OD/041/13 Genetically modified serotype 5/3 adenovirus coding for granulocyte macrophage colony-stimulating factor, EMA/OD/190/13 Doxorubicin(6-maleimidocaproyl)hydrazone, EMA/OD/037/16 16-base single-stranded peptide nucleic acid oligonucleotide linked to a 7 aminoacid peptide, EMA/OD/266/14 Olaratumab, EMA/OD/159/15 Glucopyranosyl lipid A stable emulsion and recombinant New York esophageal squamous cell carcinoma-1 protein, EMA/OD/184/15 (S)-N-(5-((R)-2-(2,5-difluorophenyl)pyrrolidin-1-yl)pyrazolo[1,5-a]pyrimidin-3-yl)-3-hydroxypyrrolidine-1-carboxamide hydrogen sulfate, EMA/OD/215/15 Human/murine chimeric monoclonal antibody against endoglin, EMA/OD/238/15 Sindbis virus envelope pseudotyped lentiviral vector encoding New York esophageal squamous cell carcinoma-1 protein, EMA/OD/064/16 Autologous CD4+ and CD8+ T cells transduced with lentiviral vector containing an affinity-enhanced T-cell receptor targeting the New York esophageal antigen-1

Designations withdrawn: EMEA/OD/059/03 N-acetylsarcosyl-glycyl-L-valyl-D-alloisoleucyl-L-threonyl-L-norvalyl-L-isoleucyl-L-arginyl-L-prolyl-N-ethylamide, EMEA/OD/050/05 (1R, 2R, 4S)-4-{(2R)-2-[(3S,6R,7E,9R,10R,12R,14S,15E,17E,19E,21S,23S,26R, 27R,34aS)-9,27-dihydroxy-10,21-dimethoxy-6,8,12,14,20,26-hexamethyl-1,5,11,28,29-pentaoxo-1,4,5,6,9,10,11,12,13,14,21,22,23,24,25,26,27,28,29,31,32,33,34,34a-tetra-cosahydro-3H-23,27-epoxypyrido[2,1-c][1,4]oxazacyclohentriacontin-3-yl]propyl}-2-methoxy-cyclohexyldimethyl-phosphinate, EMEA/OD/071/05 Brostallicin , EMEA/OD/083/06 Fenretinide , EMEA/OD/044/08 Palifosfamide , EMA/OD/141/10 Ombrabulin , EMA/OD/110/11 (1-methyl-2-nitro-1H-imidazole-5-yl)methyl N,N'-bis(2-bromoethyl) diamidophosphate

2.2.15. - EMA/OD/147/16

Treatment of acute myeloid leukemia

Action: For adoption

Documents tabled:
Draft Summary report

Notes:
There have been 48 designations for this condition: Please see 2.1.12.

2.2.16. - EMA/OD/157/16

Treatment of ovarian cancer

Action: For adoption

Documents tabled:
Draft Summary report

Notes:
There have been 31 designations for this condition: Please see 2.2.13.

2.2.17. - EMA/OD/065/16

Treatment of noninsulinoma pancreatogenous hypoglycaemia syndrome

Action: For adoption

Documents tabled:
Draft Summary report

2.2.18. - EMA/OD/131/16

Treatment of non-cutaneous mature T/NK-cell lymphoma

Action: For adoption

Documents tabled:
Draft Summary report

2.2.19. - EMA/OD/154/16

Treatment of leukocyte adhesion deficiency type I

Action: For adoption

Documents tabled:
Draft Summary report

2.2.20. - EMA/OD/136/16

Treatment of cutaneous T-cell lymphoma

Action: For adoption

Documents tabled:
Draft Summary report

Notes:
There have been 13 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

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

2.2.21. - EMA/OD/135/16

Treatment of acute myeloid leukaemia

Action: For adoption

Documents tabled:
Draft Summary report

Notes:
There have been 48 designations for this condition: Please see 2.1.12.

2.2.22. - EMA/OD/160/16

Treatment of Merkel cell carcinoma

Action: For adoption

Documents tabled:
Draft Summary report

Notes:
There has been 1 designation for this condition: EMA/OD/150/15 Recombinant human monoclonal IgG1 antibody against programmed death ligand-1

Designation withdrawn: EMEA/OD/155/09 Maytansinoid-conjugated humanised monoclonal antibody against CD56

2.2.23. - EMA/OD/083/16

Prevention of short bowel syndrome

Action: For adoption

Documents tabled:
Draft Summary report

2.2.24. - EMA/OD/127/16

Treatment of Smith-Magenis syndrome

Action: For adoption

Documents tabled:
Draft Summary report

2.2.25. - EMA/OD/145/16

Treatment of glioma

Action: For adoption

Documents tabled:
Draft Summary report

Notes:
There have been 42 designations for this condition: EMEA/OD/004/02 Pseudomonas exotoxin (domains II/III)-Interleukin 13 chimeric protein, EMEA/OD/037/02 Iodine (131I) anti-nucleohistone H1 chimeric biotinylated monoclonal antibody , 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 limnot null and st, 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

Designations withdrawn: EMEA/OD/067/01 Carmustine (solution for intratumoral injection), EMEA/OD/074/01 Human transferrin conjugated to mutant diptheria toxin, EMEA/OD/067/03 Cilengitide , EMEA/OD/050/06 Iodine (131I) anti-tenascin monoclonal antibody 81C6 , 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

2.2.26. - EMA/OD/138/16

Treatment of acute pancreatitis

Action: For adoption

Documents tabled:
Draft Summary report

Notes:
There has been 1 designation for this condition: EMA/OD/072/14 Ulinastatin

2.2.27. - EMA/OD/137/16

Treatment of opioid poisoning

Action: For adoption

Documents tabled:
Draft Summary report

2.2.28. - EMA/OD/140/16

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

2.2.29. - EMA/OD/155/16

Treatment of acute myeloid leukaemia

Action: For adoption

Documents tabled:
Draft Summary report

Notes:
There have been 48 designations for this condition: Please see 2.1.12.

2.2.30. - EMA/OD/114/16

Treatment of sudden sensorineural hearing loss

Action: For adoption

Documents tabled:
Draft Summary report

2.2.31. - EMA/OD/161/16

Treatment of Duchenne muscular dystrophy

Action: For adoption

Documents tabled:
Draft Summary report

Notes:
There have been 24 designations for this condition: Please see 2.1.14.

2.2.32. - EMA/OD/164/16

Treatment of Mucopolysaccharidosis type IIIA (Sanfilippo A syndrome)

Action: For adoption

Documents tabled:
Draft Summary report

Notes:
There have been 5 designations for this condition: Please see 2.2.12.

2.2.33. - EMA/OD/144/16

Treatment of sickle cell disease

Action: For adoption

Documents tabled:
Draft Summary report

Notes:
There have been 10 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, 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

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

2.2.34. - EMA/OD/150/16

Treatment of haemophagocytic lymphohistiocytosis

Action: For adoption

Documents tabled:
Draft Summary report

Notes:
There has been 1 designation for this condition: EMEA/OD/153/09 Recombinant human anti-interferon gamma monoclonal antibody

2.2.35. - EMA/OD/143/16

Diagnosis of glioma

Action: For adoption

Documents tabled:
Draft Summary report

Notes:
There have been 3 designations for this condition: EMEA/OD/015/06 4-[123I] iodo-L-phenylalanine, EMA/OD/280/14 Fluciclovine (18F), EMA/OD/201/15 Florilglutamic acid (18F)

2.2.36. - EMA/OD/153/16

Treatment of coenzyme Q10 deficiency syndrome

Action: For adoption

Documents tabled:
Draft Summary report

2.2.37. - EMA/OD/159/16

Treatment of ovarian cancer

Action: For adoption

Documents tabled:
Draft Summary report

Notes:
There have been 31 designations for this condition: Please see 2.2.13.

2.2.38. - EMA/OD/162/16

Treatment of diffuse large B-Cell lymphoma

Action: For adoption

Documents tabled:
Draft Summary report

Notes:
There have been 8 designations for this condition: EMEA/OD/091/08 Recombinant hisitidine-tagged idiotype immunoglobulin Fab fragment of clonal B-cell receptors , EMA/OD/160/10 Lenalidomide , EMA/OD/116/13 Ibrutinib , EMA/OD/092/14 obinutuzumab, EMA/OD/215/14 Humanised Fc engineered monoclonal antibody against CD19,

EMA/OD/005/15 Humanised anti-CD37 monoclonal antibody conjugated to maytansinoid DM1, EMA/OD/016/16 3-(5-amino-2-methyl-4-oxoquinazolin-3(4H)-yl)piperidine-2,6-dione hydrochloride, EMA/OD/084/15 2'-deoxyguanosylyl-(3',5'-phosphoryl)-2'-deoxythymidylyl-(3',5'-phosphoryl)- 2'-deoxyguanosylyl-(3',5'-phosphoryl)-2'-deoxycytidylyl-(3',5'-phosphoryl)-2'-deoxycytidylyl -(3',5'-phosphoryl)-2'-deoxycytidylyl-(3',5'-phosphoryl)-2'-deoxyguanosdrome (cinca)

Designation withdrawn: EMEA/OD/126/09 Pixantrone dimaleate

2.3. Revision of the COMP opinions

None

2.4. COMP opinions adopted via written procedure following previous meeting

None

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 6-8 September 2016 COMP meeting

2.7. Evaluation on-going

Twenty four applications for orphan designation will not be discussed as evaluation is on-going**.**

Action: For information

Notes:
Cross reference to other agenda point. See 6.8.1. Table 6. Evaluation Ongoing.

3. Requests for protocol assistance with significant benefit question

3.1. Ongoing procedures

3.1.1. -

Treatment of soft tissue sarcoma

Action: For discussion

3.1.2. -

Treatment of tuberculosis

Action: For discussion

3.2. Finalised letters

3.2.1. -

Treatment of microscopic polyangiitis

Action: For information

3.2.2. -

Treatment of granulomatosis with polyangiitis

Action: For information

3.3. New requests

3.3.1. -

Treatment in haematopoietic stem cell transplantation

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. Onivyde - irinotecan - EMA/OD/051/11, EU/3/11/933, EMEA/H/C/004125

Baxter Innovations GmbH; Treatment of pancreatic cancer

Action: For adoption, Oral explanation to be held on 7 September 2016 at time 15:00

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

Notes:
Status of the procedure at the CHMP: Opinion adopted in July 2016

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

4.2.1. – olaratumab – EMA/OD/266/14, EU/3/15/1447, EMEA/H/C/004216

Eli Lilly Nederland B.V.; Treatment of soft tissue sarcoma

Action: For discussion

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

4.2.2. - obeticholic acid – EMEA/OD/073/09, EU/3/10/753, EMEA/H/C/004093

Intercept Italia s.r.l; Treatment of primary biliary cirrhosis

Action: For discussion

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

4.2.3. - edotreotide – EMA/OD/219/14, EU/3/15/1450, EMEA/H/C/004140

Advanced Accelerator Applications; Diagnosis of gastro-entero-pancreatic neuroendocrine tumours

Action: For discussion

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

4.2.4. - venetoclax – EMA/OD/124/12, EMEA/H/C/004106, EU/3/12/1080

AbbVie Ltd.; Treatment of chronic lymphocytic leukaemia

Action: For discussion

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

4.2.5. - cediranib - EMEA/H/C/004003, EU/3/14/1303, EMA/OD/059/14

AstraZeneca AB; Treatment of ovarian cancer

Action: For discussion

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

4.2.6. NINLARO - ixazomib - EMEA/H/C/003844, EU/3/12/1060, EMA/OD/110/12

Takeda Pharma A/S; Multiple myeloma

Action: For discussion

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

Notes:
Status of the procedure at the CHMP: Re-examination of opinion adopted in May 2016

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

NPS Pharma Holdings Limited; Treatment of hypoparathyroidism

Action: For information, no oral explanation

4.2.8. - 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 information, oral explanation postponed

4.3. On-going procedures

Action: For information

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

None

6. Organisational, regulatory and methodological matters

6.1. Mandate and organisation of the COMP

6.1.1. Protocol Assistance Working Group

Proposed meeting time on 7 September 2016 at time 08:00, room 2G (To be confirmed)

Document(s) tabled:
Draft agenda
Draft minutes from July meeting

6.1.2. COMP Drafting Group

Proposed meeting time on 8 September 2016 at time 08:00, room 2G (To be confirmed)

6.1.3. Preclinical Models Working Group

Proposed meeting time on 8 September 2016 at time 18:30, room 2G (To be confirmed)

6.1.4. Recommendation on criteria for competence and expertise of COMP members

Action: For information

Document(s) tabled:
Briefing note
Annex B to nomination invitation letters to MS
Expertise of COMP members

6.2. Coordination with EMA Scientific Committees or CMDh-v

6.2.1. PDCO/COMP Working Group

Proposed meeting time on 7 September 2016 at time 14:00, room 2G (To be confirmed)

6.2.2. Recommendations on eligibility to PRIME – report from CHMP

Action: For information

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

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

6.3.1. Patients' and Consumers' Organisations (PCWP) and Healthcare Professionals' Organisations (HCPWP) joint meetings

EMA workshop with patient and healthcare professional representatives about communication on medicines - 8 March 2016

EMA Human Scientific Committees 'Working Parties with Patients' and Consumers' Organisations (PCWP) and Healthcare Professionals' Organisations (HCPWP) joint meeting - 9 March 2016

EMA Human Scientific Committees 'Working Parties with Patients' and Consumers' Organisations (PCWP) and Healthcare Professionals' Organisations (HCPWP) joint meeting – Workshop on social media – 19 September 2016

EMA Human Scientific Committees 'Working Parties with Patients' and Consumers' Organisations (PCWP) and Healthcare Professionals' Organisations (HCPWP) joint meeting – 20 September 2016

Action: For information

Document(s) tabled:
Report of a joint EMA workshop with patient and healthcare professional representatives about communication on medicines - 8 March 2016 (EMA/194543/2016)
Minutes of the PCWP and HCPWP joint meeting - 9 March 2016 (EMA/183905/2016
Agenda – PCWP and HCPWP joint meeting – Workshop on social media – 19 September 2016 (EMA/825257/2015)
Draft Agenda – PCWP and HCPWP joint meeting – 20 September 2016 (EMA/428004/2016)

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

EMA Human Scientific Committees' Working Parties with Patients' and Consumers' Organisations (PCWP) meeting – 14 June 2016

EMA Human Scientific Committees' Working Parties with Patients' and Consumers' Organisations (PCWP) 10th Anniversary meeting – 14 June 2016

Action: For information

Document(s) tabled:
Agenda – PCWP meeting – 14 June (EMA/ 274681/2016)
Minutes - PCWP meeting – 14 June 2016 (EMA/419205/2016)
Agenda - PCWP 10th Anniversary meeting – 14 June 2016 (EMA/ 392315 /2016)

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

EMA Human Scientific Committees' Working Parties with Healthcare Professionals' Organisations (HCPWP) meeting – 15 June

Action: For information

Document(s) tabled:
Agenda - HCPWP meeting – 15 June (EMA/285607/2016)
Minutes HCPWP meeting – 15 June (EMA/418148/2016)

6.4. Cooperation within the EU regulatory network

6.4.1. European Commission

None

6.5. Cooperation with International Regulators

6.5.1. Food and Drug Administration (FDA)

None

6.5.2. Japanese Pharmaceuticals and Medical Devices Agency (PMDA)

None

6.5.3. The Therapeutic Goods Administration (TGA), Australia

None

6.5.4. Health Canada

None

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

None

6.7. COMP work plan

6.7.1. COMP Work Plan 2016

Action: For information

Document(s) tabled:

COMP Work Plan 2016
6-7-1 COMP Work plan tracking tool 2016

6.7.2. COMP Work Plan 2017

Action: For information

Document(s) tabled:
COMP draft Work Plan 2017

6.8. Planning and reporting

6.8.1. List of all applications submitted/expected and the COMP coordinatorship distribution of valid applications submitted in 2016

Action: For information

6.8.2. Overview of orphan marketing authorisations/applications

Action: For information

7. Any other business

7.1. EMA organisational adjustments presentation

Action: For information

7.2. EMA Business Pipeline activity and Horizon scanning

Action: For information

7.3. EMA Workshop on scientific and regulatory challenges of genetically modified cell-based cancer immunotherapy products

The workshop will take place on 15-16 November 2016 at the EMA.

Action: For information

Notes:
More information available on the following links:
http://www.ema.europa.eu/docs/en_GB/document_library/Agenda/2016/08/WC500212061.pdf
http://www.ema.europa.eu/docs/en_GB/document_library/Template_or_form/2016/08/WC500212063.docx

7.4. COMP Workshop on Defining orphan conditions

The workshop will take place on 9 December 2016 at the EMA.

Action: For information

Document(s) tabled:
Draft agenda