25 August 2015
EMA/COMP/572401/2015
Procedure Management and Committees Support Division

Committee for Orphan Medicinal Products (COMP)

Draft agenda for the meeting on 1-3 September 2015

Chair: Bruno Sepodes – Vice-Chair: Lesley Greene

01 September 2015, 09:00-19:30, room 2F

02 September 2015, 08:30-18:30, room 2F

03 September 2015, 08:30-13:00, 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 1-3 September 2015. See September 2015 COMP minutes (to be published post October 2015 COMP meeting).

1.2. Adoption of agenda

COMP agenda for 1-3 September 2015.

1.3. Adoption of the minutes

COMP minutes for 14-16 July 2015.

2. Applications for orphan medicinal product designation

2.1. For opinion

2.1.1. - EMA/OD/062/15

Treatment of snakebite envenomation

Action: For adoption, Oral explanation to be held on 1 September 2015 at time 09:30

Documents tabled:
Draft Summary report with response to LoQs

2.1.2. - EMA/OD/083/15

Treatment of cervical cancer

Action: For adoption, Oral explanation to be held on 1 September 2015 at time 14:30

Documents tabled:
Draft Summary report with response to LoQs

2.1.3. - EMA/OD/079/15

Treatment of retinal artery occlusion

Action: For adoption, Oral explanation to be held on 1 September 2015 at time 15:30

Documents tabled:
Draft Summary report with response to LoQs
Reader's guidance

2.1.4. - EMA/OD/077/15

Treatment of mantle cell lymphoma

Action: For adoption, Oral explanation to be held on 1 September 2015 at time 17:00

Documents tabled:
Draft Summary report with response to LoQs

Notes:
There have been 7 designations for this condition: EMEA/OD/053/03 Recombinant antibody derivative against human CD19 and CD3, EMEA/OD/064/04 Recombinant histidine-tagged idiotype immunoglobulin Fab fragment of clonal B-cell receptors, EMEA/OD/058/06 Temsirolimus, EMA/OD/059/10 (3S)-3-{4-[7-(aminocarbonyl)-2H-indazol-2-yl] phenyl} piperidine tosylate monohydrate salt, EMA/OD/113/10 Autologous tumour-derived immunoglobulin idiotype coupled to keyhole limpet haemocyanin, EMA/OD/078/11 Lenalidomide, EMA/OD/171/12 1-[(3R)-3-[4-amino-3-(4-phenoxyphenyl)-1H- pyrazolo [3,4-d]pyrimidin-1-yl]-1-piperidinyl]-2-propen-1-one

2.1.5. - EMA/OD/078/15

Treatment of primary mediastinal large B-cell lymphoma

Action: For adoption, Oral explanation to be held on 1 September 2015 at time 17:00

Documents tabled:
Draft Summary report with response to LoQs

2.1.6. - EMA/OD/066/15

Treatment of Dravet Syndrome

Action: For information

Document tabled:
Withdrawal request 066-15

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

2.1.7. - EMA/OD/065/15

Treatment of Lennox-Gastaut Syndrome

Action: For information

Document tabled:
Withdrawal request 065-15

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

2.1.8. - EMA/OD/073/15

Treatment of pulmonary hypertension associated with idiopathic interstitial pneumonia

Action: For adoption, Oral explanation to be held on 2 September 2015 at time 11:00

Documents tabled:
Draft Summary report with response to LoQs

2.1.9. - EMA/OD/071/15

Treatment of primary graft dysfunction following lung transplantation

Action: For adoption, Oral explanation to be held on 2 September 2015 at time 12:00

Documents tabled:
Draft Summary report with response to LoQs

2.1.10. - EMA/OD/088/15

Treatment of aneurysmal subarachnoid hemorrhage (aSAH)

Action: For adoption, Oral explanation to be held on 2 September 2015 at time 14:30

Documents tabled:
Draft Summary report with response to LoQs

Notes:
There has been 1 designation for this condition: sodium nitrite EMA/OD/131/13

2.1.11. - EMA/OD/067/15

Treatment of progressive supranuclear palsy

Action: For adoption, Oral explanation to be held on 2 September 2015 at time 15:30

Documents tabled:
Draft Summary report with response to LoQs

Notes:
There have been 2 designations for this condition: EMA/OD/076/10 Methylthioninium, EMA/OD/261/14 N-(3-(4-(3-(diisobutylamino)propyl)piperazin-1-yl)propyl)-1H-benzo[d]imidazol-2-amine disulphate salt
Designations withdrawn: EMEA/OD/074/09 4-benzyl-2-naphtalen-1-yl-1,2,4-thiadiazolidine-3,5-dione, EMEA/OD/129/09 Davunetide

2.1.12. - EMA/OD/075/15

Treatment of hereditary angioedema

Action: For adoption, Oral explanation to be held on 2 September 2015 at time 17:00

Documents tabled:
Draft Summary report with response to LoQs

Notes:

There has been 1 designation for this condition: EMA/OD/170/14 3-[2-(4-carbamimidoyl-phenylcarbamoyl)-5-methoxy-4-vinyl-phenyl]-6-(cyclopropylmethyl-carbamoyl)-pyridine-2-carboxylic acid

2.1.13. - EMA/OD/092/15

Treatment of chronic iron overload requiring chelation therapy

Action: For adoption, Oral explanation to be held on 3 September 2015 at time 09:30

Documents tabled:
Draft Summary report with response to LoQs

Notes:
There have been 2 designations for this condition: EMEA/OD/061/01 4-(3,5-bis(hydroxy-phenyl)-1,2,4) triazol-1-yl) benzoic acid, EMEA/OD/008/09 (S)-3'-(OH)-desazadesferrithiocin-polyether, magnesium salt
Designation withdrawn: EMEA/OD/060/03 4,5-dihydro-2-(2,4-dihydroxyphenyl)-4-methylthiazole-4(S)-carboxylic acid

2.2. For discussion / preparation for an opinion

2.2.1. - EMA/OD/105/15

Treatment of systemic sclerosis

Action: For adoption

Documents tabled:
Draft Summary report
COMP coordinator's comments

Notes:
There have been 12 designations for this condition: EMEA/OD/032/01 Halofuginone hydrobromide, EMEA/OD/035/05 Peptide 144 TGF-beta1-inhibitor (TSLDASIIWAMMQN), EMEA/OD/079/08 Type I native bovine skin collagen, EMEA/OD/106/08 Treprostinil diethanolamine, EMA/OD/095/10 Paquinimod, EMA/OD/143/12 2-[4-Methoxy-3-(2-m-tolyl-ethoxy)-benzoylamino]-indan-2-carboxylic acid, EMA/OD/153/12 Terguride, EMA/OD/044/14 Riociguat, EMA/OD/129/14 1-(6-benzothiazolylsulfonyl)-5-chloro-1H-indole-2-butanoic acid, EMA/OD/148/14 Humanized IgG1 monoclonal antibody against human eotaxin-2, EMA/OD/225/14 Nitroglycerin, EMA/OD/296/14 Autologous adipose tissue-derived stromal vascular fraction cells
Designations withdrawn: EMEA/OD/051/01 Human engineered monoclonal antibody specific for Transforming Growth Factor ß1, EMA/OD/163/11 Pomalidomide, EMA/OD/156/12 Terguride

2.2.2. - EMA/OD/118/15

Treatment of hepatocellular carcinoma

Action: For adoption

Documents tabled:

Draft Summary report
COMP coordinator's comments

Notes:
There have been 18 designations for this condition: EMEA/OD/015/02 Thymalfasin, EMEA/OD/087/04 Pegylated arginine deiminase, EMEA/OD/048/04 Doxorubicine polyisohexylcyanoacrylate nanoparticles, EMEA/OD/018/05 Nemorubicin hydrochloride, EMEA/OD/109/05 Sorafenib tosylate, EMEA/OD/090/07 N-[4-(3-amino-1H-indazol-4 yl)phenyl]-N'-(2-fluoro-5-methylphenyl) urea, EMEA/OD/070/09 NGR-human tumour necrosis factor, EMEA/OD/076/09 Vaccinia GM-CSF/TK-deactivated virus, EMA/OD/065/10 (S)-10-[(dimethylamino)methyl]-4-ethyl-9-hydroxy-4-O-[alpha-(2", 4", 5", 7"-tetranitro-9"-fluorenylideneaminooxy)propionyl]-1H-pyrano[3', 4', 6', 7']indolizino[1,2-beta]-quinoline-3, 14-(4H, 12H)-dione, hydrochloride, EMA/OD/096/10 Doxorubicin hydrochloride (in heat-sensitive liposomes), EMA/OD/170/10 Sulfonated monophosphorylated mannose oligosaccharide, EMA/OD/003/11 Peretinoin, EMA/OD/045/11 Resminostat, EMA/OD/031/12 Ramucirumab, EMA/OD/159/12 4-[2-(6-methylpyridin-2-yl)-5,6-dihydro-4H-pyrrolo[1,2-b]pyrazol-3-yl]-quinoline-6-carboxamide monohydrate, EMA/OD/115/13 Tivantinib , EMA/OD/160/14 Diaspirin cross-linked haemoglobin, EMA/OD/087/15 2-(2-phenylvinyl)-4-[4-methylpiperazin-1-yl)]-6-(5-methyl-2H-pyrazol-3-yl-amino)-pyrimidine L(+) tartrate salt
Designations withdrawn: EMEA/OD/013/01 Seocalcitol, EMEA/OD/026/02 Doxorubicin carbon/iron magnetically targeted microparticles, EMEA/OD/032/03 Nolatrexed, EMEA/OD/046/07 4-[3,5-bis(trimethylsilyl)benzamido] benzoic acid, EMA/OD/075/11 Brivanib alaninate

2.2.3. - EMA/OD/072/15

Treatment of idiopathic pulmonary fibrosis

Action: For adoption

Documents tabled:
Draft Summary report
COMP coordinator's comments

Notes:
There have been 12 designations for this condition: EMEA/OD/033/04 Heparin-Sodium, EMEA/OD/052/04 Pirfenidone, EMEA/OD/054/07 Interferon gamma, EMEA/OD/105/07 Recombinant human monoclonal antibody against transforming growth factor beta-1, 2 and 3, EMEA/OD/104/09 Macitentan, EMA/OD/079/10 2-(2-chlorophenyl)-4-[3-(dimethylamino)phenyl]-5-methyl-1H-pyrazolo[4,3-C]pyridine-3,6(2H,5H)-dione, EMA/OD/091/11 4-[[9-[(3S)-tetrahydro-3-furanyl]-8-[(2,4,6-trifluorophenyl)amino]-9H-purin-2-yl]amino]-trans-cyclohexanol, EMA/OD/048/12 Recombinant human pentraxin-2, EMA/OD/111/12 Tralokinumab, EMA/OD/186/12 nintedanib, EMA/OD/051/14 Humanised anti-alpha v beta 6 monoclonal antibody, EMA/OD/130/14 1-(6-benzothiazolylsulfonyl)-5-chloro-1H-indole-2-butanoic acid
Designations withdrawn: EMEA/OD/002/05 Interferon gamma, EMA/OD/029/10 Ambrisentan, EMEA/OD/075/04 Acetylcysteine, EMEA/OD/027/08 Bosentan

2.2.4. - EMA/OD/115/15

Treatment of focal segmental glomerulosclerosis

Action: For adoption

Documents tabled:
Draft Summary report

Notes:
There has been 1 designation for this condition: EMA/OD/146/10 Fresolimumab

2.2.5. - EMA/OD/111/15

Treatment of immune thrombocytopenia

Action: For adoption

Documents tabled:
Draft Summary report
COMP coordinator's comments

2.2.6. - EMA/OD/110/15

Treatment of aniridia

Action: For adoption

Documents tabled:
Draft Summary report

2.2.7. - EMA/OD/108/15

Treatment of chronic lymphocytic leukaemia/small lymphocytic lymphoma

Action: For adoption

Documents tabled:
Draft Summary report
COMP coordinator's comments

Notes:
There have been 2 designations for this condition: EMA/OD/022/14 Humanised Fc engineered monoclonal antibody against CD19, EMA/OD/078/14 Selinexor
Designation withdrawn: EMA/OD/056/13 Idelalisib

2.2.8. - EMA/OD/107/15

Treatment of acute lymphoblastic leukaemia

Action: For adoption

Documents tabled:
Draft Summary report
COMP coordinator's comments

Notes:
There have been 19 designations for this condition: EMEA/OD/046/01 2-chloro-9-[2-deoxy-2-fluoro-ß-D-arabinofuranosyl]adenine, EMEA/OD/032/08 Pegylated L-asparaginase,

EMEA/OD/063/04 L-Asparaginase, EMEA/OD/015/05 Nelarabine, EMEA/OD/074/05 Dasatinib, EMEA/OD/033/06 L-asparaginase encapsulated in erythrocytes, EMEA/OD/070/06 Forodesine hydrochloride, EMEA/OD/065/07 Mercaptopurine (oral liquid), EMEA/OD/064/07 Methotrexate (oral liquid), EMEA/OD/002/08 Vincristine sulphate liposomes, EMEA/OD/114/08 Mercaptopurine (oral suspension), EMA/OD/097/10 Recombinant human histone H1.3 and recombinant human N-bis-met-histone H1.3, EMEA/OD/029/09 Blinatumomab, EMEA/OD/084/09 6-thioguanine (oral liquid), EMEA/OD/122/09 Benzamide, 3-(2-imidazo[1,2-b]pyridazin-3-ylethynyl)-4-methyl-N-[4-[(4-methyl-1-piperazinyl)methyl]-3-(trifluoromethyl)phenyl], EMA/OD/168/10 Pegylated recombinant Erwinia chrysanthemi L-asparaginase, EMA/OD/001/11 Allogeneic T cells encoding an exogenous thymidine kinase gene, EMA/OD/143/13 (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/120/14 Allogeneic CD34+ cells expanded ex-vivo with an aryl hydrocarbon receptor antagonist
Designations withdrawn: EMEA/OD/022/03 Aplidine, EMEA/OD/038/05 Imatinib mesilate, EMEA/OD/067/08 Allogeneic ex vivo expanded umbilical cord blood cells

2.2.9. - EMA/OD/098/15

Treatment nasopharyngeal carcinoma

Action: For adoption

Documents tabled:
Draft Summary report
COMP coordinator's comments

2.2.10. - EMA/OD/113/15

Treatment of glioma

Action: For adoption

Documents tabled:
Draft Summary report

Notes:
There have been 40 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/023/08 Topotecan hydrochloride (liposomal), EMEA/OD/034/08 Gadodiamide (liposomal), 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/050/06 Iodine (131I) anti-tenascin monoclonal antibody 81C6, 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/104/08 Autologous tumour-derived gp96

heat shock protein-peptide complex, EMEA/OD/098/09 Recombinant fusion protein consisting of the extracellular portion of CD95 fused to the Fc part of a human IgG1 molecule, EMA/OD/086/10 7-beta-hydroxycholesteryl-3-beta-oleate, EMA/OD/092/12 IL-12-secreting dendritic cells, loaded with autologous tumour lysate, EMA/OD/077/11 L-cysteine, L-leucyl-L-alpha-glutamyl-L-alpha-glutamyl-L-lysyl-L-lysylglycyl-L-asparaginyl-L-tyrosyl-L-valyl-L-valyl-L-threonyl-L-alpha-aspartyl-L-histidyl-S-[1-[(4-carboxycyclohexyl)methyl]-2,5-dioxo-3-pyrrolidinyl]-complex with keyhole limdinator_Applica, EMA/OD/050/11 2-hydroxyoleic acid, EMA/OD/157/11 Adenovirus-associated vector containing human Fas-c gene, EMA/OD/019/12 Doxorubicin (administered after synthetic double-stranded siRNA oligonucleotide directed against claudin-5 complexed with polyethyleneimine), EMA/OD/170/12 4-[2-(6-methylpyridin-2-yl)-5,6-dihydro-4H-pyrrolo[1,2-b]pyrazol-3-yl]-quinoline-6-carboxamide monohydrate, EMA/OD/148/12 1,2:5,6-Dianhydrogalactitol, EMA/OD/136/12 Synthetic double-stranded siRNA oligonucleotide directed against Claudin-5 complexed with polyethyleneimine (prior to administration of doxorubicin), EMA/OD/086/13 Autologous ex vivo expanded leukocytes treated with 5-aza-2'-deoxycytidine, EMA/OD/001/14 Autologous dendritic cells pulsed with RNA from glioma stem cells, EMA/OD/107/13 Allogeneic and autologous haptenised and irradiated cells and cell lysates derived from glioma, EMA/OD/174/13 Autologous dendritic cells pulsed with tumour antigen-derived synthetic peptides (MAGE-1, HER-2, AIM-2, TRP-2, gp-100, and interleukin-13 receptor alpha), EMA/OD/111/14 Recombinant human bone morphogenetic protein 4, EMA/OD/003/14 Paclitaxel-succinate- Arg-Arg-Leu-Ser-Tyr-Ser-Arg-Arg-Arg-Phe, EMA/OD/065/14 Humanised recombinant monoclonal antibody against epidermal growth factor receptor conjugated to maleimidocaproyl monomethylauristatin F, EMA/OD/132/14 Olaptesed pegol, EMA/OD/200/14 5,5'-(4-(trifluromethyl)benzylazanediyl)bis(methylene)diquinolin-8-ol, EMA/OD/159/14 Chloroquine, EMA/OD/176/14 Adenovirus serotype 5 containing partial E1A deletion and an integrin-binding domain, EMA/OD/251/14 Recombinant human glutamate oxaloacetate transaminase 1

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, EMA/OD/031/10 Glutathione-pegylated liposomal doxorubicin hydrochloride, 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/049/12 Humanised monoclonal antibody against epidermal growth factor receptor

2.2.11. - EMA/OD/120/15

Treatment of acute myeloid leukaemia

Action: For adoption

Documents tabled:
Draft Summary report
COMP coordinator's comments

Notes:
There have been 39 designations for this condition: EMEA/OD/022/00 Gemtuzumab ozogamicin, EMA/OD/044/10 Allogeneic T cells encoding an exogenous TK gene, EMEA/OD/028/04 Midostaurin, EMEA/OD/051/04 Homoharringtonine, 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/056/06 Antisense oligonucleotide 5'-d[P-Thio] (CCCTG CTCCC CCCTG GCTCC)-3' (see comments box for cenersen sodium), 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/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/105/12 Liposomal daunorubicin, 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/258/14 Ulocuplumab, 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

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

2.2.12. - EMA/OD/119/15

Prevention of graft-versus-host disease

Action: For adoption

Documents tabled:
Draft Summary report

Notes:
There have been 5 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

2.2.13. - EMA/OD/091/15

Treatment of adrenal insufficiency

Action: For adoption

Documents tabled:
Draft Summary report

Notes:
There have been 3 designations for this condition: EMEA/OD/009/03 Prasterone, EMEA/OD/108/05 Hydrocortisone (modified release tablet), EMEA/OD/095/06 Hydrocortisone (modified release tablet)

2.2.14. - EMA/OD/117/15

Treatment of intestinal malabsorption in pre-term infants

Action: For adoption

Documents tabled:
Draft Summary report

2.2.15. - EMA/OD/093/15

Treatment of Middle East respiratory syndrome

Action: For adoption

Documents tabled:
Draft Summary report
COMP coordinator's comments

2.2.16. - EMA/OD/002/15

Treatment of narcolepsy

Action: For adoption

Documents tabled:
Draft Summary report

Notes:
There have been 2 designations for this condition: EMEA/OD/087/06 1-{3-[3-(4-chlorophenyl)propoxy]propyl}piperidine, hydrochloride, EMA/OD/254/14 Mazindol
Designation withdrawn: EMEA/OD/051/02 Sodium oxybate

2.2.17. - EMA/OD/109/15

Treatment of Duchenne muscular dystrophy

Action: For adoption

Documents tabled:
Draft Summary report
COMP coordinator's comments

Notes:
There have been 22 designations for this condition: EMA/OD/142/11 Exon 45 specific phosphorothioate oligonucleotide, EMA/OD/143/11 Exon 53 specific phosphorothioate oligonucleotide, 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-dimethylamdinator_Application.Appl, 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/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
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

2.2.18. - EMA/OD/097/15

Treatment of idiopathic hypersomnia

Action: For adoption

Documents tabled:
Draft Summary report
COMP coordinator's comments

2.2.19. - EMA/OD/096/15

Treatment of achromatopsia caused by mutations in the CNGA3 gene

Action: For adoption

Documents tabled:
Draft Summary report
COMP coordinator's comments

2.2.20. - EMA/OD/069/15

Treatment of glioma

Action: For adoption

Documents tabled:
Draft Summary report

Notes:
There have been 40 designations for this condition: please see agenda point 2.2.10
Designations withdrawn: please see agenda point 2.2.10

2.2.21. - EMA/OD/064/15

Treatment of blastic plasmacytoid dendritic cell neoplasm

Action: For adoption

Documents tabled:
Draft Summary report
COMP coordinator's comments

2.2.22. - EMA/OD/112/15

Treatment of acute myeloid leukaemia

Action: For adoption

Documents tabled:

Draft Summary report

Notes:
There have been 39 designations for this condition: please see agenda point 2.2.11
Designations withdrawn: please see agenda point 2.2.11

2.2.23. - EMA/OD/100/15

Treatment of tuberous sclerosis

Action: For adoption

Documents tabled:
Draft Summary report

Notes:
There has been 1 designation for this condition: EMA/OD/010/10 Everolimus

2.2.24. - EMA/OD/102/15

Treatment for Ebola virus disease

Action: For adoption

Documents tabled:
Draft Summary report

Notes:
There have been 2 designations for this condition: EMA/OD/310/14 Rintatolimod, EMA/OD/250/14 Fibrinogen-coated albumin spheres

2.2.25. - EMA/OD/081/15

Treatment of diffuse large B-cell lymphoma

Action: For adoption

Documents tabled:
Draft Summary report
COMP coordinator's comments

Notes:
There have been 6 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
Designation withdrawn: EMEA/OD/126/09 Pixantrone dimaleate

2.3. Amendment of an existing orphan drug designation

2.3.1. Sialic acid – EMA/OD/126/11

Ultragenyx UK Limited - United Kingdom; Treatment of hereditary inclusion body myopathy

Action: For adoption

Document tabled:
Revised draft Summary report

2.4. COMP opinions adopted via written procedure following previous meeting

2.4.1. Lanreotide acetate - EMA/OD/027/15

Prof. Dr R.T.Gansevoort; Treatment of autosomal dominant polycystic kidney disease

Action: For information

2.5. Appeal

2.5.1. Autologous human peripheral blood Vdelta1+ T lymphocytes activated in vitro by cytokine and monoclonal antibody treatment – EMA/OD/006/15

Lymphact - Lymphocyte Activation Technologies S.A. - Portugal; Treatment of chronic lymphocytic leukaemia/ small lymphocytic lymphoma

Action: For adoption, Oral explanation to be held on 1 September 2015 at time 18:00

Documents tabled:
Revised draft Summary report
Sponsor's grounds for appeal

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 1-3 September 2015 COMP meeting

2.7. Evaluation on-going

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

Action: For information

Notes:
Cross reference to other agenda point. See 5.8.1.

3. Requests for protocol assistance with significant benefit question

3.1. Ongoing procedures

3.1.1. -

Treatment of sickle cell disease

Action: For adoption

3.1.2. -

Treatment of systemic sclerosis

Action: For adoption

3.1.3. -

Treatment of acromegaly

Action: For adoption

3.2. Finalised letters

3.2.1. -

Treatment of graft-versus-host disease

Action: For information

3.2.2. -

Treatment of Urea Cycle Disorders:

a) treatment of ornithine transcarbamylase deficiency ;

b) treatment of carbamoyl-phosphate synthase-1 deficiency ;

c) treatment of citrullinaemia type 1 ;

d) treatment of argininosuccinic aciduria ;

e) treatment of hyperargininaemia ;

f) treatment of N-acetylglutamate synthetase (NAGS) deficiency ;

g) treatment of citrullinaemia type 2 ;

h) treatment of ornithine translocase deficiency (hyperornithinaemia-hyperammonaemia homocitrullinuria (HHH) syndrome) .

Action: For information

3.3. New requests

3.3.1. -

Treatment of ovarian cancer

Action: For information

3.3.2. -

Treatment of Prader-Willi syndrome

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. Cresemba - isavuconazole – EMEA/H/C/002734

Basilea Medical Ltd;

a) treatment of invasive aspergillosis (EMA/OD/009/14, EU/3/14/1284)

b) treatment of mucormycosis (EMA/OD/010/14, EU/3/14/1276)

Action: For adoption, Oral explanation to be held on 1 September 2015 at time 11:00

Documents tabled:
Draft report on review of OMPD
CHMP AR

Notes:
Status of the procedure at the CHMP: CHMP adopted positive opinion in July 2015.

4.1.2. Obizur - susoctocog alfa – EMEA/H/C/002792, EMA/OD/043/10, EU/3/10/784

Baxter AG; Treatment of haemophilia A

Action: For adoption, Oral explanation to be held on 1 September 2015 at time 12:00

Documents tabled:
Draft report on review of OMPD
CHMP AR

Notes:
Status of the procedure at the CHMP: CHMP adopted positive opinion in July 2015.

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

4.2.1. – blinatumomab - EMA/OD/029/09, EU/3/09/650, EMEA/H/C/003731

Amgen Europe B.V.; Treatment of acute lymphoblastic leukaemia

Action: For discussion

Document tabled:
Draft report on review of OMPD

4.2.2. - Efmoroctocog alfa – EMA/OD/030/10, EU/3/10/783, EMEA/H/C/003964

Biogen Idec Ltd; Treatment of haemophilia A

Action: For discussion

Document tabled:
Draft report on review of OMPD

4.2.3. - Carfilzomib – EMEA/OD/120/07, EU/3/08/548, EMEA/H/C/003790

Amgen Europe B.V.; Treatment of multiple myeloma

Action: For discussion

Document tabled:
Draft report on review of OMPD

4.2.4. - Lumacaftor / ivacaftor – EMA/OD/032/14, EU/3/14/1333, EMEA/H/C/003954

Vertex Pharmaceuticals (U.K.) Ltd.; Treatment of cystic fibrosis

Action: For discussion

Document tabled:
Draft report on review of OMPD

4.2.5. - Glyceryl tri-(4-phenylbutyrate) – EMEA/H/C/003822

Horizon Therapeutics Limited;

a) treatment of carbamoyl-phosphate synthase-1 deficiency (EMA/OD/124/09, EU/3/10/733)

b) treatment of ornithine carbamoyltransferase deficiency (EMA/OD/002/10, EU/3/10/734)

c) treatment of citrullinaemia type 1 (EMA/OD/003/10, EU/3/10/735)

d) treatment of argininosuccinic aciduria (EMA/OD/004/10, EU/3/10/736)

e) treatment of hyperargininaemia (EMA/OD/005/10, EU/3/10/737)

f) treatment of ornithine translocase deficiency (hyperornithinaemia-hyperammonaemia homocitrullinuria (HHH) syndrome) (EMA/OD/006/10, EU/3/10/738)

g) treatment of citrullinaemia type 2 (EMA/OD/007/10, EU/3/10/739

Action: For discussion

Document tabled:
Draft report on review of OMPD

4.3. On-going procedures

4.3.1. List of on-going procedures

Action: For information

4.4. COMP opinions adopted via written procedure following previous meeting

4.4.1. Strensiq - asfotase alfa - EMEA/H/C/003794, EMA/OD/071/08, EU/3/08/594

Alexion Europe SAS; Treatment of hypophosphatasia

Action: For information

5. Organisational, regulatory and methodological matters

5.1. Mandate and organisation of the COMP

5.1.1. Strategic Review & Learning meetings

COMP/PDCO Strategic Review & Learning Meeting under the Luxembourg Presidency to be held on 15-16 October 2015 in Bonn

Action: For discussion

5.1.2. Election of Chair and Vice-Chair – 6 October 2015

Action: the nominations and candidates' résumés in support of their candidature should be forwarded

Documents tabled:
COMP Rules of Procedure EMEA/COMP/8212/00 Rev. 3
Procedure for the election of the COMP chairperson and vice-chairperson

5.2. Coordination with EMA Scientific Committees or CMDh-v

None.

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

5.3.1. Significant Benefit Working Group

Proposed meeting time 3 September at 08:30-09:30, room: 2F

Action: For information

Documents tabled:

Draft SBWG agenda for 3 September 2015
SBWG minutes for 16 July 2015

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

EMA Human Scientific Committees' Working Parties with Patients' and Consumers' Organisations (PCWP) and Healthcare Professionals' Organisations (HCPWP) joint meeting Workshop on risk minimisation measures - 16 Sept 2015

EMA Human Scientific Committees' Working Parties with Patients' and Consumers' Organisations (PCWP) and Healthcare Professionals' Organisations (HCPWP) joint meeting – 04 June 2015

EMA Human Scientific Committees' Working Party with Healthcare Professionals' Organisations (HCPWP) meeting – 04 June 2015

EMA Human Scientific Committees' Working Party with Patients' and Consumers' Organisations (PCWP) meeting – 03 June 2015

Action: For information

Documents tabled:
Draft PCWP and HCPWP joint workshop on risk minimisation measures agenda
Minutes of the PCWP/HCPWP joint meeting – 04 June 2015
Minutes of the HCPWP meeting – 04 June 2015
Minutes of the PCWP plenary meeting – 03 June 2015

5.3.3. Biologics Working Party (BWP)

Action: For information

5.4. Cooperation within the EU regulatory network

5.4.1. European Commission

On the occasion of the 50th anniversary of the adoption of the first EU legislation on human medicines, the European Commission is organising a conference on "50 Years of EU Pharma legislation: Achievements and future perspectives", that will take place on 28 September 2015 in Brussels, at the Charlemagne Building, Rue de la Loi 170.

Action: For information

Document tabled:

Conference Programme

Note: You can find more information on the Conference at:
http://ec.europa.eu/health/human-use/events/ev_20150928_en.htm

5.4.2. Review of the 2003 Communication on Orphan Medicinal Products

Action: For discussion

5.5. Cooperation with International Regulators

5.5.1. Food and Drug Administration (FDA)

EMA/FDA teleconference on Orphan Medicines – 21 July 2015

Action: for information

Document tabled:
Agenda

5.5.2. Update on the status of the IMI2 project ADAPT-SMART

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

None.

5.7. COMP work plan

None.

5.8. Planning and reporting

5.8.1. List of all applications submitted/expected and the COMP coordinatorship distribution of valid applications submitted in 2015

Action: For information

5.8.2. Overview of orphan marketing authorisations/applications

Action: For information

5.8.3. Meeting dates

Action: For information

Documents tabled:
COMP meeting dates for 2016-2018
Committee meeting dates 2016-2018

6. Any other business

None.