3 October 2016
EMA/COMP/646954/2016
Inspections, Human Medicines Pharmacovigilance and Committees Division

Committee for Orphan Medicinal Products (COMP)

Draft agenda for the meeting on 04-06 October 2016

Chair: Bruno Sepodes – Vice-Chair: Lesley Greene

04 October 2016, 09:00-20:00, room 2F

05 October 2016, 08:30-20:00, room 2F

06 October 2016, 08:30-15:00, 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 4-6 October 2016. See October 2016 COMP minutes (to be published post November 2016 COMP meeting).

1.2. Adoption of agenda

COMP agenda for 4-6 October 2016.

1.3. Adoption of the minutes

COMP minutes for 6-8 September 2016.

2. Applications for orphan medicinal product designation

2.1. For opinion

2.1.1. - EMA/OD/137/16

Treatment of opioid poisoning

Action: For adoption, Oral explanation to be held on 4 October 2016 at time 09:30

Documents tabled:
Draft Summary report with response to LoQs

2.1.2. - EMA/OD/160/16

Treatment of Merkel cell carcinoma

Action: For adoption, Oral explanation to be held on 4 October 2016 at time 12:00

Documents tabled:
Draft Summary report with response to LoQs

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

2.1.3. - EMA/OD/144/16

Treatment of sickle cell disease

Action: For adoption, Oral explanation to be held on 6 October 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/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

2.1.4. - EMA/OD/149/16

Prevention of graft-versus-host disease

Action: For adoption, Oral explanation to be held on 4 October 2016 at time17:30

Documents tabled:
Draft Summary report with response to LoQs

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

2.1.5. - EMA/OD/145/16

Treatment of glioma

Action: For adoption, Oral explanation to be held on 5 October 2016 at time 10:00

Documents tabled:
Draft Summary report with response to LoQs

Notes:
There have been 44 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 , EMA/OD/067/16 Zoledronic acid , EMA/OD/085/16 Temozolomide

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

2.1.6. - EMA/OD/103/16

Treatment of ovarian cancer

Action: For adoption, Oral explanation to be held on 5 October 2016 at time 11:00

Documents tabled:
Draft Summary report with response to LoQs

Notes:
There have been 30 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/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/094/11 Vincaleukoblastin-23-oic acid, O4-deacetyl-2-[(2-mercaptoethoxy)carbonyl]hydrazide, disulfide with …, EMA/OD/002/12 1-(4-{4-amino-7-[1-(2-hydroxyethyl)-1H- pyrazol-4-yl] thieno [3,2-c]pyridin-3-yl}phenyl)-3-(3-fluorophenyl)urea

2.1.7. - EMA/OD/157/16

Treatment of ovarian cancer

Action: For adoption, Oral explanation to be held on 5 October 2016 at time 11:00

Documents tabled:
Draft Summary report with response to LoQs

Notes:
There have been 30 designations for this condition: Please see 2.1.6.

2.1.8. - EMA/OD/140/16

Treatment of spinal cord injury

Action: For adoption, Oral explanation to be held on 5 October 2016 at time 14:00

Documents tabled:
Draft Summary report with response to LoQs

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

2.1.9. - EMA/OD/083/16

Prevention of short bowel syndrome

Action: For information

Documents tabled:
Withdrawal request of 19 September 2016

2.1.10. - EMA/OD/159/16

Treatment of ovarian cancer

Action: For adoption, Oral explanation to be held on 5 October 2016 at time 16:30

Documents tabled:
Draft Summary report with response to LoQs

Notes:
There have been 30 designations for this condition: Please see 2.1.6.

2.1.11. - EMA/OD/114/16

Treatment of sudden sensorineural hearing loss

Action: For adoption, Oral explanation to be held on 4 October 2016 at time 11:00

Documents tabled:
Draft Summary report with response to LoQs

2.1.12. - 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, Oral explanation to be held on 5 October 2016 at time 18:30

Documents tabled:
Draft Summary report with response to LoQs

2.1.13. - EMA/OD/138/16

Treatment of acute pancreatitis

Action: For adoption, Oral explanation to be held on 6 October 2016 at time 09:00

Documents tabled:
Draft Summary report with response to LoQs

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

2.1.14. - EMA/OD/139/16

Treatment of IgA nephropathy

Action: For adoption, Oral explanation to be held on 6 October 2016 at time 10:00

Documents tabled:
Draft Summary report with response to LoQs

2.1.15. - EMA/OD/132/16

Treatment of X-linked adrenoleukodystrophy

Action: For adoption

Documents tabled:
Draft Summary report with response to LoQs

2.1.16. - EMA/OD/133/16

Treatment of Crigler Najjar syndrome

Action: For adoption

Documents tabled:
Draft Summary report with response to LoQs

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

2.1.17. - EMA/OD/151/16

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

Action: For adoption

Documents tabled:
Draft Summary report with response to LoQs

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

2.1.18. - EMA/OD/162/16

Treatment of diffuse large B-Cell lymphoma

Action: For adoption

Documents tabled:
Draft Summary report with response to LoQs

Notes:
There have been 7 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

Designations withdrawn: EMEA/OD/126/09 Pixantrone dimaleate, 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'-deoxyguanosylyl-(3',5'-phosphoryl)-2'-deoxycytidilyl-(3',5'-phosphoryl)-2'-deoxycytidylyl-(3',5'-phosphoryl)-2'-deoxycytidylyl-(3',5'-phosphoryl)-2'-deoxycytidylyl-(3',5'-phosphoryl)-2'-deoxythymidylyl-(3',5'-phosphoryl)-2'-deoxyadenosyl-(3',5'-phosphoryl)-2'-deoxycytidylyl-(3',5'-phosphoryl)-2'-deoxyguanosylyl-(3',5'-phosphoryl)-2'-deoxycytidylyl-(3',5'-phosphoryl)-2'-deoxyguanosylyl-(3',5'-phosphoryl)-2'-deoxycytidylyl-(3',5'-phosphoryl)-2'-deoxyadenosyl-(3',5'-phosphoryl)-2'-deoxycytidylyl-(3',5'-phosphoryl)-2'-deoxyguanosylyl-2'-deoxycytidylyl-(3',5'-phosphoryl)-2'-deoxyadenosyl-(3',5'-phosphoryl)-2'-deoxycytidine, sodium salt

2.1.19. - EMA/OD/135/16

Treatment of acute myeloid leukaemia

Action: For information

Documents tabled:
Withdrawal request of 14 September 2016

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 Ig-like receptors (1-7 F9), EMEA/OD/020/06 Lestaurtinib, EMEA/OD/024/07 Arsenic trioxide, EMEA/OD/069/07 Amonafide L-malate, EMEA/OD/060/08 2-[[3-({4-[(5-{2-[(3-Fluorophenyl)amino]-2-oxoethyl}-1H-pyrazol-3-yl)amino]-quinazolin-7-yl}oxy)propyl](ethyl)amino]ethyl dihydrogen phosphate trihydrate, 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

2.1.20. - EMA/OD/136/16

Treatment of cutaneous T-cell lymphoma

Action: For information

Documents tabled:
Withdrawal request of 14 September 2016

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. For discussion / preparation for an opinion

2.2.1. - EMA/OD/181/16

Treatment of plasma cell myeloma

Action: For adoption

Documents tabled:
Draft Summary report

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

2.2.2. - EMA/OD/062/16

Treatment of poisoning by local anesthetics

Action: For adoption

Documents tabled:
Draft Summary report

2.2.3. - EMA/OD/187/16

Treatment of sickle cell disease

Action: For adoption

Documents tabled:
Draft Summary report

Notes:
There have been 10 designations for this condition: Please see 2.1.3.

2.2.4. - EMA/OD/168/16

Treatment of glycogen storage disease type Ia

Action: For adoption

Documents tabled:
Draft Summary report

2.2.5. - EMA/OD/165/16

Treatment of retinitis pigmentosa

Action: For adoption

Documents tabled:
Draft Summary report

Notes:
There have been 17 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 , EMA/OD/102/16 Adenovirus associated viral vector serotype 5 containing the human RPGR 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/175/16

Treatment of facioscapulohumeral muscular dystrophy

Action: For adoption

Documents tabled:
Draft Summary report

Notes:
There has been 1 designation for this condition: EMA/OD/268/14 505 amino acid protein, corresponding to amino acids 2-506 of the wild type human histidyl-tRNA synthetase

2.2.7. - EMA/OD/177/16

Treatment of recurrent *Clostridium difficile* infection

Action: For adoption

Documents tabled:
Draft Summary report

2.2.8. - EMA/OD/208/16

Treatment 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

2.2.9. - EMA/OD/174/16

Treatment of facioscapulohumeral muscular dystrophy

Action: For adoption

Documents tabled:
Draft Summary report

Notes:
There has been 1 designation for this condition: EMA/OD/268/14 505 amino acid protein, corresponding to amino acids 2-506 of the wild type human histidyl-tRNA synthetase

2.2.10. - EMA/OD/170/16

Treatment of gastric cancer

Action: For adoption

Documents tabled:
Draft Summary report

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

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

2.2.11. - EMA/OD/180/16

Treatment of smallpox infection

Action: For adoption

Documents tabled:
Draft Summary report

2.2.12. - EMA/OD/169/16

Treatment of tenosynovial giant cell tumour, localised and diffuse type

Action: For adoption

Documents tabled:
Draft Summary report

Notes:
There has been 1 designation for this condition: EMA/OD/279/14 [5-(5-chloro-1H-pyrrolo[2,3-b]pyridin-3-ylmethyl)-pyridin-2-yl]-(6-trifluoromethyl-pyridin-3-ylmethyl)-amine hydrochloride

2.2.13. - EMA/OD/163/16

Treatment of congenital adrenal hyperplasia

Action: For adoption

Documents tabled:
Draft Summary report

Notes:
There has been 1 designation for this condition: EMEA/OD/020/05 Hydrocortisone (modiefied release tablet)

Designation withdrawn: EMA/OD/063/15 Verucerfont

2.2.14. - EMA/OD/186/16

Treatment of fibrodysplasia ossificans progressiva

Action: For adoption

Documents tabled:
Draft Summary report

Notes:
There has been 1 designation for this condition: EMA/OD/145/14 Palovarotene

2.2.15. - EMA/OD/178/16

Treatment of graft-versus-host disease

Action: For adoption

Documents tabled:
Draft Summary report

Notes:

There have been 11 designations for this condition: EMEA/OD/038/00 Inolimomab, EMEA/OD/046/05 A mixture of anti-CD3 mAb (SPV-T3a)-ricin A chain fusion protein and anti-CD7 mAb (WT1)-ricin A chain fusion protein, EMEA/OD/009/06 Methoxsalen, EMEA/OD/049/06 Budesonide (oral use), EMEA/OD/068/06 Ex-vivo cultured adult human mesenchymal stem cells, EMA/OD/022/10 Murine monoclonal antibody against CD26, EMA/OD/197/12 Allogeneic bone marrow derived mesenchymal cells expanded ex vivo in synthetic media , EMA/OD/267/14 Human plasma-derived alpha-1 proteinase inhibitor , EMA/OD/017/16 Rimiducid, EMA/OD/110/16 Cannabidiol , EMA/OD/118/16 Recombinant humanised monoclonal antibody against human complement component C5a

Designation withdrawn: EMEA/OD/020/00 Thalidomide

2.2.16. - EMA/OD/173/16

Treatment of facioscapulohumeral muscular dystrophy

Action: For adoption

Documents tabled:
Draft Summary report

Notes:
There has been 1 designation for this condition: EMA/OD/268/14 505 amino acid protein, corresponding to amino acids 2-506 of the wild type human histidyl-tRNA synthetase

2.2.17. - EMA/OD/061/16

Treatment of hyperphenylalaninemia

Action: For adoption

Documents tabled:
Draft Summary report

Notes:
There have been 3 designations for this condition: EU/3/03/163 5,6,7,8-Tetrahydrobiopterin, EU/3/04/199 Tetrahydrobiopterin, EU/3/09/708 Pegylated recombinant phenylalanine ammonia lyase

Designations withdrawn: EU/3/05/308 Sapropterin

2.2.18. - EMA/OD/166/16

Treatment of angiosarcoma

Action: For adoption

Documents tabled:
Draft Summary report

2.2.19. - EMA/OD/183/16

Treatment of aromatic L-amino acid decarboxylase (AADC) deficiency

Action: For adoption

Documents tabled:
Draft Summary report

2.2.20. - EMA/OD/184/16

Treatment of argininosuccinic aciduria

Action: For adoption

Documents tabled:
Draft Summary report

Notes:
There have been 4 designations for this condition: EMA/OD/004/10 glyceryl tri-(4-phenybutyrate), EMA/OD/107/10 Human heterologous liver cells (for infusion), EMA/OD/059/13 Heterologous human adult liver-derived progenitor cells , EMA/OD/124/15 Sodium benzoate

2.2.21. - EMA/OD/185/16

Treatment of N-acetylglutamate synthase deficiency

Action: For adoption

Documents tabled:
Draft Summary report

Notes:
There has been 1 designation for this condition: EMA/OD/061/13 Heterologous human adult liver-derived progenitor cells

2.2.22. - EMA/OD/152/16

Treatment of Huntington's disease

Action: For adoption

Documents tabled:
Draft Summary report

Notes:
There have been 6 designations for this condition: EMA/OD/070/14 Cysteamine bitartrate , EMA/OD/169/14 5-bromo-N-(prop-2-yn-1-yl)-2-(1H-1,2,4-triazol-1-yl) pyrimidine-4,6-diamine, EMA/OD/256/14 Chimeric 2'-O-(2-methoxyethyl) modified oligonucleotide targeted to huntingtin RNA , EMA/OD/311/14 Phenol, 4-[2-(aminomethyl)-4-thiazolyl]-2,6-bis(1,1-dimethylethyl) monohydrochloride , EMA/OD/325/14 AASSGVSTPGSAGHDIITEQPRS , EMA/OD/017/15 5,7-dichloro-2-dimethylaminomethyl-8-hydroxyquinoline hydrochloride

2.2.23. - EMA/OD/134/16

Treatment of multiple myeloma

Action: For adoption

Documents tabled:

Draft Summary report

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

2.2.24. - EMA/OD/188/16

Treatment of pulmonary arterial hypertension

Action: For adoption

Documents tabled:
Draft Summary report

Notes:
There have been 3 designations for this condition: EMEA/OD/018/08 Beraprost sodium, EMA/OD/023/11 Macitentan, EMA/OD/111/11 Sodium nitrite

2.2.25. - EMA/OD/172/16

Treatment of Huntington's disease

Action: For adoption

Documents tabled:
Draft Summary report

Notes:
There have been 6 designations for this condition: EMA/OD/070/14 Cysteamine bitartrate , EMA/OD/169/14 5-bromo-N-(prop-2-yn-1-yl)-2-(1H-1,2,4-triazol-1-yl) pyrimidine-4,6-diamine, EMA/OD/256/14 Chimeric 2'-O-(2-methoxyethyl) modified oligonucleotide targeted to huntingtin RNA , EMA/OD/311/14 Phenol, 4-[2-(aminomethyl)-4-thiazolyl]-2,6-bis(1,1-

dimethylethyl) monohydrochloride , EMA/OD/325/14 AASSGVSTPGSAGHDIITEQPRS , EMA/OD/017/15 5,7-dichloro-2-dimethylaminomethyl-8-hydroxyquinoline hydrochloride

2.2.26. - EMA/OD/176/16

Treatment of facioscapulohumeral muscular dystrophy

Action: For adoption

Documents tabled:
Draft Summary report

Notes:
There has been 1 designation for this condition: EMA/OD/268/14 505 amino acid protein, corresponding to amino acids 2-506 of the wild type human histidyl-tRNA synthetase

2.3. Amendment of the COMP opinions

2.3.1. Recombinant human acid sphingomyelinase – EMEA/OD/004/01, EU/3/01/056

Genzyme Europe BV; Treatment of Niemann-Pick disease, type B

Action: For adoption

Document tabled:
Amended draft Summary report

Notes:
Opinion adopted in February 2001

2.4. COMP opinions adopted via written procedure following previous meeting

2.4.1. Carbamazepine - EMA/OD/148/16

University of Newcastle upon Tyne; Treatment of metaphyseal chondrodysplasia, Schmid type

COMP coordinator: Ingeborg Barisic

Action: For information

Document tabled:
Summary report

2.4.2. P-ethoxy growth factor receptor-bound protein 2 (Grb2) antisense oligonucleotide - EMA/OD/155/16

Clinical Network Services (UK) Ltd; Treatment of acute myeloid leukaemia

COMP coordinator: Frauke Naumann-Winter

Action: For information

Document tabled:
Summary report

2.4.3. Ubiquinol - EMA/OD/153/16

Centro de Investigación Biomédica en Red (CIBER); Treatment of coenzyme Q10 deficiency syndrome

COMP coordinator: Irena Bradinova/Pauline Evers

Action: For information

Document tabled:
Summary report

2.5. Appeal

2.5.1. Naltrexone – EMA/OD/035/16

Able AB; Treatment of fibromyalgia

Action: For adoption, Oral explanation to be held on 4 October 2016 at time 15:00

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

Notes:
Negative opinion adopted in July 2016

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 4-6 October 2016 COMP meeting

2.7. Evaluation on-going

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

Action: For information

Notes:
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 tuberculosis

Action: For adoption

3.1.2. -

Treatment in haematopoietic stem cell transplantation

Action: For adoption

3.2. Finalised letters

3.2.1. -

Treatment of soft tissue sarcoma

Action: For information

3.3. New requests

None

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

AstraZeneca AB; Treatment of ovarian cancer

Action: For information, oral explanation cancelled.

4.1.2. Ninlaro - ixazomib - EMEA/H/C/003844, EU/3/11/899, EMA/OD/048/11

Takeda Pharma A/S; Treatment of multiple myeloma

Action: For adoption, Oral explanation to be held on 4 October 2016 at time 16:30

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

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

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

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

Action: For adoption, Oral explanation to be held on 6 October 2016 at time 11:00

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

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

4.1.4. Lartruvo – 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 adoption

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

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

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

4.2.1. - 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 adoption, Oral explanation to be held on 4 October 2016 at time 14:00

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

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

AbbVie Ltd.; Treatment of chronic lymphocytic leukaemia

Action: For adoption, Oral explanation to be held on 5 October 2016 at time 09:00

Documents tabled:
Draft report on review of OMPD

4.2.3. - 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 adoption, Oral explanation to be held on 5 October 2016 at time 12:00

Documents tabled:
Draft report on review of OMPD

4.2.4. - EMA/OD/036/08, EU/3/08/578, EMEA/H/C/003769

Orphan Europe S.A.R.L.; Treatment of cystinosis

Action: For information

Documents tabled:
Draft report on review of OMPD

4.3. On-going procedures

Action: For information

4.4. Public Summary of Opinion

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. Strategic Review & Learning meetings

COMP Strategy Review & Learning meetings, 17-18 October 2016, Rome, Italy

Action: For information

Document tabled:
Draft agenda

6.1.2. Protocol Assistance Working Group

Proposed meeting time on 5 October 2016 at time 08:00, room 2H (To be confirmed)

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

6.1.3. COMP Drafting Group

Proposed meeting time on 6 October 2016 at time 08:00, room 3H (To be confirmed)

6.1.4. Preclinical Models Working Group

None

6.1.5. Recommendations on eligibility to PRIME – report from CHMP

Action: For information

Document(s) tabled:

PRIME eligibility requests - list of adopted outcomes September 2016

6.1.6. Process for communication of applications from the EMA and the company to COMP members

Action: For information

6.1.7. Significant Benefit Working Group

Proposed meeting time on 4 October 2016 at time 13:00, function room

6.2. Coordination with EMA Scientific Committees or CMDh-v

6.2.1. PDCO/COMP Working Group

Proposed meeting time on 5 October 2016 at 13:00, (To be confirmed)

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

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

None

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

None

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)

Action: For information

Document tabled:
Draft Agenda September 13 2016

Notes:
Monthly teleconference

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

None