11 July 2017
EMA/COMP/391757/2017
Inspections, Human Medicines Pharmacovigilance and Committees Division

Committee for Orphan Medicinal Products (COMP)

Draft agenda for the meeting on 11-13 July 2017

Chair: Bruno Sepodes – Vice-Chair: Lesley Greene

11 July 2017, 09:00-19:00, room 2F

12 July 2017, 08:30-19:00, room 2F

13 July 2017, 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

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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 11-13 July 2017. See July 2017 COMP minutes (to be published post September 2017 COMP meeting).

1.2. Adoption of agenda

COMP agenda for 11-13 July 2017.

1.3. Adoption of the minutes

COMP minutes for 13-15 June 2017.

2. Applications for orphan medicinal product designation

2.1. For opinion

2.1.1. - EMA/OD/054/17

Treatment of spinal cord injury

Action: For information

Document(s) tabled:
Withdrawal request of 4 July 2017

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Designation withdrawn: EMEA/OD/041/08 Autologous urothelial and smooth muscle cells

2.1.2. - EMA/OD/049/17

Prevention of bronchopulmonary dysplasia

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

Document(s) tabled:
Draft Summary report with response to LoQs

Notes:
There have been 5 designations for this condition: EMA/OD/161/13 Caffeine citrate, EMA/OD/018/14 Retinol, EMA/OD/172/13 Recombinant human surfactant protein D, EMA/OD/270/14 Recombinant human club cell 10 KDa protein, EMA/OD/010/15 Allogeneic ex-vivo-expanded human umbilical cord blood-derived mesenchymal stem cells

2.1.3. - EMA/OD/056/17

Treatment of naevoid basal-cell carcinoma syndrome (Gorlin syndrome)

Action: For adoption, Oral explanation to be held on 12 July 2017 at 09:30

Document(s) tabled:
Draft Summary report with response to LoQs

Notes:
Designations withdrawn: EMEA/OD/048/09 N-[6-(cis-2,6-DimItraconazoleethylmorpholin-4-yl)pyridine-3-yl]-2-methyl-4'-(trifluoromethoxy)[1,1'-biphenyl]-3-carboxamide

2.1.4. - EMA/OD/057/17

Treatment of glioma

Action: For adoption, Oral explanation to be held on 12 July 2017 at 11:00

Document(s) tabled:
Draft Summary report with response to LoQs

Notes:
There have been 39 designations for this condition: EMEA/OD/026/03 Herpes simplex virus lacking infected cell protein 34.5, EMEA/OD/055/03 Gimatecan, EMEA/OD/050/04 Biotinylated anti-tenascin monoclonal antibody for use with 90-Yttrium, EMEA/OD/038/04 Anti epidermal growth factor receptor antibody h-R3, EMEA/OD/030/05 Oligonucleotide phosphorothioate (TAAACGTTATAACGTTATGACGTCAT), sodium salt, EMEA/OD/068/05 Enzastaurin hydrochloride, EMEA/OD/110/05 4-[131I] iodo-L-phenylalanine, EMEA/OD/081/06 Autologous dendritic cells pulsed with autologous tumour cell lysate, EMEA/OD/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 limpet haemocyanin, EMA/OD/050/11 2-hydroxyoleic acid, EMA/OD/157/11 Adenovirus-associated vector containing human Fas-c gene , 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/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, EMA/OD/215/16 5-aminolevulinic acid

Designations withdrawn: EMEA/OD/004/02 Pseudomonas exotoxin (domains II/III)-Interleukin 13 chimeric protein, EMEA/OD/074/01 Human transferrin conjugated to mutant diptheria toxin, EMEA/OD/067/01 Carmustine (solution for intratumoral injection), EMEA/OD/050/06 Iodine (131I) anti-tenascin monoclonal antibody 81C6, EMEA/OD/037/02 Iodine (131I) anti-nucleohistone H1 chimeric biotinylated monoclonal antibody, EMEA/OD/067/03 Cilengitide, EMEA/OD/050/07 Doxorubicin hydrochloride (drug eluting beads), EMEA/OD/051/07 Irinotecan hydrochloride (drug eluting beads), 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/019/12 Doxorubicin (administered after synthetic double-stranded siRNA oligonucleotide directed against claudin-5 complexed with polyethyleneimine) , EMA/OD/136/12 Synthetic double-stranded siRNA oligonucleotide directed against Claudin-5 complexed with polyethyleneimine (prior to administration of doxorubicin) , EMA/OD/113/15 Dronabinol and cannabidiol

2.1.5. - EMA/OD/036/17

Treatment of sickle cell disease

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

Document(s) tabled:
Draft Summary report with response to LoQs

Notes:
There have been 11 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/084/13 (1R,3R,4R,5S)-3-O-[2-O-benzoyl-3-O-(sodium(2S)-3-cyclohexyl-propanoate-2-yl)-β-D-galactopyranosyl]-4-O-(α-L-fucopyranosyl)-5-orothylamido-cyclohexane-1-carboxylic acid (ethyl-2-amidyl-ethyloxy-2-acetyl-(8-amino-1,3,6-naphthalene-tris sodium sulfonate) amide, EMA/OD/184/13 Autologous CD34+ haematopoietic stem cells transduced with lentiviral vector encoding the human beta A-T87Q-globin gene , EMA/OD/210/14 Sevufparin sodium, EMA/OD/187/16 2-hydroxy-6-((2-(1-isopropyl-1H-pyrazol-5-yl)pyridin-3-yl) methoxy)benzaldehyde, EMA/OD/144/16 Synthetic human hepcidin

Designations withdrawn: EMA/OD/162/12 Poloxamer 188, EMA/OD/249/14 5-hydroxymethyl-2-furfural

2.1.6. - EMA/OD/050/17

Treatment of myelodysplastic syndromes

Action: For adoption, Oral explanation to be held on 12 July 2017 at 14:30

Document(s) tabled:
Draft Summary report with response to LoQs

Notes:
There have been 7 designations for this condition: EMEA/OD/059/01 Azacitidine, EMEA/OD/059/02 Decitabine, EMEA/OD/083/03 3-(4'aminoisoindoline-1'-one)-1-piperidine-2,6-dione, EMEA/OD/014/08 Sapacitabine, EMA/OD/161/11 (E)-2,4,6-trimethoxystyryl-3-carboxymethylamino-4-methoxybenzyl-sulfone sodium salt, EMA/OD/048/14 Recombinant fusion protein consisting of a modified form of the extracellular domain of human Activin Receptor IIB linked to the human IgG1 Fc domain, EMA/OD/272/16 Humanised IgG4 monoclonal antibody to the human toll-like receptor type 2

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

2.1.7. - EMA/OD/003/17

Treatment of N-acetylglutamate synthase deficiency

Action: For information

Document(s) tabled:
Withdrawal request of 28 June 2017

Notes:
There has been 1 designation for this condition: EMA/OD/185/16 sodium benzoate

2.1.8. - EMA/OD/043/17

Treatment of mucopolysaccharidosis type VI (Maroteaux-Lamy syndrome)

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

Document(s) tabled:
Draft Summary report with response to LoQs

Notes:
There has been 1 designation for this condition: EMA/OD/147/10 Adeno-associated viral vector containing the human ARSB gene

2.1.9. - EMA/OD/044/17

Treatment of lung allograft dysfunction associated with lung transplantation

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

Document(s) tabled:

Draft Summary report with response to LoQs

2.1.10. - EMA/OD/327/16

Treatment in cardiopulmonary by-pass

Action: For adoption, Oral explanation to be held on 13 July 2017 at 09:00

Document(s) tabled:
Draft Summary report with response to LoQs

2.1.11. - EMA/OD/053/17

Treatment of primary mitochondrial myopathy

Action: For adoption, Oral explanation to be held on 13 July 2017 at 11:00

Document(s) tabled:
Draft Summary report with response to LoQs

2.1.12. - EMA/OD/052/17

Treatment of hepatocellular carcinoma

Action: For adoption

Document(s) tabled:
Draft Summary report with response to LoQs

Notes:
There have been 19 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/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/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/287/14 Lenvatinib, EMA/OD/087/15 2-(2-phenylvinyl)-4-[4-methylpiperazin-1-yl)]-6-(5-methyl-2H-pyrazol-3-yl-amino)-pyrimidine L(+) tartrate salt, EMA/OD/118/15 2-chloro-N6-(3-iodobenzyl)adenosine-5'-N-methyluronamide, EMA/OD/072/16 Mifamurtide

Designations withdrawn: EMEA/OD/013/01 Seocalcitol, EMEA/OD/026/02 Doxorubicin carbon/iron magnetically targeted microparticles, EMEA/OD/032/03 Nolatrexed, EMEA/OD/090/07 N-[4-(3-amino-1H-indazol-4 yl)phenyl]-N'-(2-fluoro-5-methylphenyl) urea, EMEA/OD/046/07 4-[3,5-bis(trimethylsilyl)benzamido] benzoic acid, EMA/OD/075/11 Brivanib alaninate, EMA/OD/031/12 Ramucirumab

2.2. For discussion / preparation for an opinion

2.2.1. - EMA/OD/079/17

Treatment of primary sclerosing cholangitis

Action: For discussion/adoption

Document(s) tabled:
Draft Summary report

Notes:
There have been 5 designations for this condition: EMA/OD/127/13 (4R,5R)-1-[[4-[[4-[3,3-dibutyl-7-(dimethylamino)-2,3,4,5-tetrahydro-4-hydroxy-1,1-dioxido-1-benzothiepin-5-yl]phenoxy]methyl]phenyl]methyl]-4-aza-1-azoniabicyclo[2.2.2]octane chloride, EMA/OD/136/13 Obeticholic acid, EMA/OD/026/14 Norursodeoxycholic acid, EMA/OD/288/14 Recombinant human monoclonal antibody binding to vascular adhesion protein-1, EMA/OD/139/15 Variant of recombinant human fibroblast growth factor 19

2.2.2. - EMA/OD/084/17

Treatment of Wilson's disease

Action: For discussion/adoption

Document(s) tabled:
Draft Summary report

Notes:
There have been 5 designations for this condition: EMEA/OD/043/03 Trientine dihydrochloride, EMEA/OD/114/07 Ammonium tetrathiomolybdate, EMA/OD/142/12 Choline tetrathiomolybdate, EMA/OD/001/15 Trientine tetrahydrochloride, EMA/OD/114/15 Adeno-associated viral vector serotype 8 encoding the human ATP7B gene under the control of the human alpha-1 antitrypsin promoter

2.2.3. - EMA/OD/094/17

Treatment of retinitis pigmentosa

Action: For discussion/adoption

Document(s) tabled:
Draft Summary report

Notes:
There have been 21 designations for this condition: EMEA/OD/057/06 4,7,10,13,16,19-Docosahexaenoic acid, EMEA/OD/043/07 Adenovirus associated viral vector serotype 4 containing the human RPE65 gene, EMEA/OD/087/08 Recombinant human proinsulin, EMA/OD/162/10 9-cis-Retinyl acetate, EMA/OD/159/11 Encapsulated human retinal pigment epithelial cell line transfected with plasmid vector expressing human ciliary neurotrophic factor, EMA/OD/006/12 Recombinant human methionine proinsulin, EMA/OD/025/13 Expanded human allogeneic neural retinal progenitor cells extracted from neural retina, EMA/OD/015/13 Recombinant human nerve growth factor, EMA/OD/031/13 Adenovirus associated viral vector serotype 5 containing the human pde6β gene,

EMA/OD/289/14 Sodium 3-[(4aR,6R,7R,7aS)-7-hydroxy-2-oxido-2-sulfanylidene-4a,6,7,7a-tetrahydro-4H-furo[3,2-d][1,3,2]dioxaphosphinin-6-yl]-2-bromo-6-phenyl-5H-imidazo[1,2-a]purin-9-one, EMA/OD/271/14 Myriocin, EMA/OD/327/14 Recombinant human mesencephalic astrocyte-derived neurotrophic factor, EMA/OD/040/15 Adenovirus-associated viral vector serotype 2 containing the human RPE65 gene, EMA/OD/158/16 Adeno-associated viral vector serotype 2/2 containing a gene encoding the channelrhodopsin-2 protein, EMA/OD/213/15 Allogeneic fetal human retinal progenitor cells expanded ex vivo, EMA/OD/208/15 4-[(2E)-1-oxo-3-(2,6,6-trimethyl-1-cyclohexen-1-yl)-2-propen-1-yl]-1-piperazinecarboxamide, EMA/OD/028/16 Adeno-associated viral vector serotype 2.7m8 containing the ChrimsonR-tdTomato gene, EMA/OD/102/16 Adenovirus associated viral vector serotype 5 containing the human RPGR gene, EMA/OD/146/16 Adeno-associated viral vector serotype 5 containing the human RLBP1 gene, EMA/OD/165/16 Adeno-associated viral vector serotype 8 encoding engineered rhodopsin DNA-binding repressor and human rhodopsin expression cassettes, EMA/OD/280/16 Antisense oligonucleotide targeting the USH2A 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

2.2.4. - EMA/OD/076/17

Treatment of infantile spasms

Action: For discussion/adoption

Document(s) tabled:
Draft Summary report

2.2.5. - EMA/OD/088/17

Treatment of growth hormone deficiency

Action: For discussion/adoption

Document(s) tabled:
Draft Summary report

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

2.2.6. - EMA/OD/097/17

Treatment of Neurodegeneration with Brain Iron Accumulation (NBIA)

Action: For discussion/adoption

Document(s) tabled:
Draft Summary report

2.2.7. - EMA/OD/090/17

Treatment of glioblastoma

Action: For discussion/adoption

Document(s) tabled:
Draft Summary report

Notes:
Designation withdrawn: EMEA/OD/012/00 Fluorouracil

2.2.8. - EMA/OD/059/17

Treatment of short bowel syndrome

Action: For discussion/adoption

Document(s) tabled:
Draft Summary report

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

2.2.9. - EMA/OD/039/17

Treatment of partial deep dermal and full thickness burns

Action: For discussion/adoption

Document(s) tabled:
Draft Summary report

Notes:
There have been 3 designations for this condition: EMEA/OD/012/02 Purified bromelain, EMA/OD/163/15 Bilayer, engineered, collagen hydrogel-based skin graft composed of autologous keratinocytes and fibroblasts, EMA/OD/068/16 Sodium hypochlorite

2.2.10. - EMA/OD/035/17

Treatment of ovarian cancer

Action: For discussion/adoption

Document(s) tabled:
Draft Summary report

Notes:
There have been 31 designations for this condition: EMEA/OD/019/02 Oregovomab, EMEA/OD/061/06 Paclitaxel (micellar), EMEA/OD/080/03 Anti-epithelial cell adhesion

molecule/anti-CD3 monoclonal antibody, EMEA/OD/044/03 Trabectedin, EMEA/OD/065/05 Imexon, EMEA/OD/063/07 Olaparib, EMEA/OD/110/07 Humanised monoclonal antibody to the folate receptor alpha, EMEA/OD/006/09 Human MHC non-restricted cytotoxic T-cell line, EMEA/OD/086/09 8-[4-(1-aminocyclobutyl)phenyl]-9-phenyl-1,2,4-triazolo[3,4-f][1,6]naphthyridin-3(2H)-one mono-hydrochloride, EMA/OD/015/10 (3S)-3-{4-[7-(aminocarbonyl)-2H-indazol-2-yl] phenyl} piperidine tosylate monohydrate salt, EMA/OD/021/10 Autologous dendritic cells pulsed with recombinant human-fusion protein (mucin 1 - glutathione S transferase) coupled to oxidised polymannose, EMA/OD/014/10 Pyr-His-Trp-Ser-Tyr-D-Lys(doxorubicinylglutarate)-Leu-Arg-Pro-Gly-NH2, acetate salt, EMA/OD/111/10 Veliparib, EMA/OD/054/11 20-pentaerythritol poly (oxy-1,2-ethanediyl)-carboxymethyl-glycinate-7-ethyl-10-hydroxycamptothecine 10-[1,4'-bipiperidine]-1'-carboxylate, EMA/OD/151/11 2-Allyl-1-[6-(1-hydroxy-1-methylethyl)pyridin-2-yl]-6-{[4-(4-methylpiperazin-1-yl)phenyl]amino}-1,2-dihydro-3H-pyrazolo[3,4-d]pyrimidin-3-one, EMA/OD/085/12 rucaparib, EMA/OD/099/12 Lurbinectedin, EMA/OD/147/12 Chimeric monoclonal antibody against claudin 6, EMA/OD/039/13 Fosbretabulin tromethamine, EMA/OD/122/13 Trebananib, EMA/OD/186/13 Genetically modified serotype 5/3 adenovirus coding for granulocyte macrophage colony-stimulating factor, EMA/OD/059/14 Cediranib, EMA/OD/281/14 Humanised anti-folate receptor 1 monoclonal antibody conjugated to maytansinoid DM4, EMA/OD/157/14 2-hydroxymethyl-2-methoxymethyl-1-azabicyclo[2,2,2]octan-3-one, EMA/OD/211/14 Chimeric group B adenovirus (11p/3) with deletions in the E3 and E4 regions, EMA/OD/223/14 N-methyl-4-({4-[({3-methyl(methylsulfonyl)amino]pyrazin-2- yl}methyl)amino]-5- (trifluoromethyl)pyrimidin-2-yl}amino)benzamide hydrochloride, EMA/OD/304/14 Human reovirus type 3 Dearing strain, EMA/OD/314/14 {2-amino-8-[4-(pyrrolidinylcarbonyl)phenyl]-(3H-benzo[f]azepin-4-yl)}-N,N-dipropylcarboxamide, EMA/OD/126/15 (5S,8S,10aR)-N-benzhydryl-5-((S)-2-(methylamino)propanamido)-3-(3-methylbutanoyl)-6-oxodecahydropyrrolo[1,2-a][1,5]diazocine-8-carboxamide, EMA/OD/159/16 Vaccine consisting of 5 survivin peptides with different human leukocyte antigen restrictions, EMA/OD/300/16 Poly-cyclodextrin-bis-cysteine-PEG3400-camptothecin-conjugate

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 N-[4-[[(2-amino-3,4-dihydro-4-oxo-6-pteridinyl)methyl]amino]benzoyl]-L-gamma-glutamyl-L-alpha-aspartyl-L-arginyl-L-alpha-aspartyl-L-alpha-aspartyl-L-cysteine, EMA/OD/002/12 1-(4-{4-amino-7-[1-(2-hydroxyethyl)-1H- pyrazol-4-yl] thieno [3,2-c]pyridin-3-yl}phenyl)-3-(3-fluorophenyl)urea, EMA/OD/114/12 Alisertib

2.2.11. - EMA/OD/068/17

Treatment of glioma

Action: For discussion/adoption

Document(s) tabled:
Draft Summary report

Notes:

There have been 39 designations for this condition: Please see 2.1.4.

2.2.12. - EMA/OD/098/17

Treatment of tuberculosis

Action: For discussion/adoption

Document(s) tabled:
Draft Summary report

Notes:
There have been 8 designations for this condition: EMEA/OD/024/05 (1R,2S) 6-bromo-alpha-[2-(dimethylamino)ethyl]-2-methoxy-alpha-(1-naphthyl)-beta-phenyl-3-quinolineethanol, EMEA/OD/036/07 N-adamantanyl-N'-Geranyl-ethylenediamine, EMEA/OD/074/07 (S)-2-nitro-6-(4-trifluoromethoxy)benzyloxy)-6,7-dihydro-5H-imidazo[2,1-b] [1,3] oxazine, EMEA/OD/094/07 (R)-2-Methyl-6-nitro-2-{4-[4-(4-trifluoromethoxyphenoxy)piperidin-1-yl]phenoxymethyl}-2,3-dihydroimidazo[2,1-b]oxazole, EMEA/OD/145/09 Rifapentine, EMA/OD/033/10 Heat-killed Mycobacterium vaccae (whole cell), EMA/OD/072/10 Para-aminosalicylic acid, EMA/OD/029/11 N-{[(5S)-3-(3-fluoro-4-thiomorpholin-4-ylphenyl)-2-oxo-1,3-oxazolidin-5-yl]methyl}acetamide

2.2.13. - EMA/OD/093/17

Treatment of Friedreich's ataxia

Action: For discussion/adoption

Document(s) tabled:
Draft Summary report

Notes:
There have been 3 designations for this condition: EMEA/OD/037/01 Idebenone, EMEA/OD/082/03 Idebenone, EMA/OD/084/11 Interferon gamma, EMA/OD/026/10 N-(6-(2-aminophenylamino)-6-oxohexyl)-4-methylbenzamide,

2.2.14. - EMA/OD/091/17

Treatment of growth hormone deficiency

Action: For discussion/adoption

Document(s) tabled:
Draft Summary report

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

2.2.15. - EMA/OD/292/16

Treatment of ATTR amyloidosis

Action: For discussion/adoption

Document(s) tabled:
Draft Summary report

Notes:
There has been 1 designation for this condition: EMA/OD/098/13 Phosphorothioate oligonucleotide targeted to transthyretin

Designation withdrawn: EMA/OD/194/13 Synthetic double-stranded siRNA oligonucleotide directed against transthyretin mRNA and covalently linked to a ligand containing three N-acetylgalactosamine residues

2.2.16. - EMA/OD/086/17

Treatment of retinitis pigmentosa

Action: For discussion/adoption

Document(s) tabled:
Draft Summary report

Notes:
There have been 21 designations for this condition: Please see 2.2.3.

2.2.17. - EMA/OD/087/17

Treatment of mastocytosis

Action: For discussion/adoption

Document(s) tabled:
Draft Summary report

Notes:
There have been 4 designations for this condition: EMEA/OD/062/04 N-(methyl-diazacyclohexyl-methylbenzamide)-azaphenyl-aminothiopyrrole, EMA/OD/016/10 Midostaurin, EMA/OD/075/14 Recombinant human diamine oxidase, EMA/OD/079/13 Cladribine

2.2.18. - EMA/OD/060/17

Treatment of Leber congenital amaurosis

Action: For discussion/adoption

Document(s) tabled:
Draft Summary report

Notes:
There have been 3 designations for this condition: EMA/OD/182/13 Adeno-associated viral vector serotype 8 containing the human GUCY2D gene, EMA/OD/163/10 9-cis-Retinyl acetate, EMA/OD/150/11 Adenovirus associated viral vector serotype 2 containing the human RPE65 gene

Designations withdrawn: EMA/OD/063/11 Adeno-associated viral vector serotype 8 containing the human AIPL1 gene

2.2.19. - EMA/OD/069/17

Treatment of glioma

Action: For discussion/adoption

Document(s) tabled:
Draft Summary report

Notes:
There have been 39 designations for this condition: Please see 2.1.4.

2.2.20. - EMA/OD/047/17

Treatment of tuberous sclerosis

Action: For discussion/adoption

Document(s) tabled:
Draft Summary report

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

2.2.21. - EMA/OD/078/17

Treatment of pancreatic cancer

Action: For discussion/adoption

Document(s) tabled:
Draft Summary report

Notes:
There have been 36 designations for this condition: EMEA/OD/055/02 G17(9) gastrin-Diphtheria Toxoid Conjugate, EMEA/OD/068/02 Rubitecan, EMEA/OD/009/05 Bovine bile extract, EMEA/OD/028/05 4-imino-1, 3-diazobicyclo-[3.1.0]-hexan-2-one, EMEA/OD/063/06 Paclitaxel (liposomal), EMEA/OD/026/06 Human telomerase reverse transcriptase peptide (611-626), EMEA/OD/103/06 Cisplatin (liposomal), EMEA/OD/100/08 L-asparaginase encapsulated in erythrocytes, EMEA/OD/006/08 Nimotuzumab, EMEA/OD/080/08 Yttrium (90Y)-DOTA-radiolabelled humanized monoclonal antibody against mucin 1, EMEA/OD/101/08 S-[2,3-bispalmitoyloxy-(2R)-propyl]-cysteinyl-GNNDESNISFKEK, EMEA/OD/030/09 Trabedersen, EMEA/OD/105/09 Brivudine, EMEA/OD/069/09 N-[(2S)-2,3-dihydroxypropyl]-3-[(2-fluoro-4-iodophenyl)amino]isonicotinamide hydrochloride, EMEA/OD/063/09 Masitinib mesilate, EMA/OD/135/10 Glufosfamide, EMA/OD/150/10 Salirasib, EMA/OD/007/11 Mixture of seven synthetic fragments consisting of p21 RAS peptides, EMA/OD/008/11 Genetically modified human adenovirus encoding human PH20 hyaluronidase, EMA/OD/051/11 Nanoliposomal irinotecan, EMA/OD/065/12 Mixture of two allogeneic human pancreatic cancer cell lines stably transduced with a retroviral vector encoding the murine alpha-(1,3)-galactosyltransferase gene, EMA/OD/037/13 (1-methyl-2-

nitro-1H-imidazole-5-yl)methyl N,N'-bis(2-bromoethyl)diamidophosphate, EMA/OD/071/13 Chimeric monoclonal antibody against claudin-18 splice variant 2, EMA/OD/164/13 Cysteamine bitartrate, EMA/OD/081/14 Immunoglobulin G1 (TEXT TOO LONG), EMA/OD/085/14 [5-Amino-1-(4-fluoro-phenyl)-1H-pyrazol-4-yl]-[3-(2,3-dihydroxy-propoxy)-phenyl]-methanone, EMA/OD/187/14 Herpes simplex type 1 virus containing cellular B-myb gene as tumour-specific promoter, EMA/OD/143/14 Heat-killed Mycobacterium obuense (whole cell), EMA/OD/173/14 Pegylated recombinant human hyaluronidase PH20, EMA/OD/302/14 Human reovirus type 3 Dearing strain, EMA/OD/034/15 Modified adenovirus serotype 5/35 containing a CMV promoter-driven transgene cassette with the human transgenes for a membrane-bound CD40 ligand (TMZ-CD40L) and full length 4-1BBL, EMA/OD/168/15 Live attenuated Listeria monocytogenes delta actA/delta inlB strain expressing human mesothelin, EMA/OD/169/15 Two allogenic irradiated pancreatic tumour cell lines, EMA/OD/193/16 Pegylated recombinant human interleukin-10, EMA/OD/241/16 Antroquinonol, EMA/OD/273/16 Humanised IgG4 monoclonal antibody to the human toll-like receptor type 2

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

2.2.22. - EMA/OD/099/17

Treatment of Pulmonary Arterial Hypertension

Action: For discussion/adoption

Document(s) tabled:
Draft Summary report

Notes:
There have been 5 designations for this condition: EMEA/OD/018/08 Beraprost sodium, EMA/OD/023/11 Macitentan, EMA/OD/111/11 Sodium nitrite, EMA/OD/179/15 Ubenimex, EMA/OD/299/16 (S)-8-{2-amino-6-[1-(5-chloro-biphenyl-2-yl)-(R)-2,2,2-trifluoro-ethoxy]-pyrimidin-4-yl}-2,8-diaza-spiro[4.5]decane-3-carboxylic acid ethyl ester)

2.2.23. - EMA/OD/085/17

Treatment of cystic fibrosis

Action: For discussion/adoption

Document(s) tabled:
Draft Summary report

Notes:
There have been 38 designations for this condition: EMEA/OD/032/00 L-Lysine-N-Acetyl-L-Cysteinate, EMEA/OD/011/03 Recombinant dog gastric lipase, EMEA/OD/038/02 Duramycin, EMEA/OD/039/04 Dexamethasone sodium phosphate encapsulated in human erythrocytes,

EMEA/OD/053/04 Alpha-1 antitrypsin (inhalation use), EMEA/OD/062/05 Mannitolum, EMEA/OD/001/06 Heparin sodium, EMEA/OD/037/09 Ciprofloxacin (liposomal), EMEA/OD/092/06 Ciprofloxacin (inhalation use), EMEA/OD/104/06 Alginate oligosaccharide (G-block) fragment, EMEA/OD/041/07 Alpha1-proteinase inhibitor (inhalation use), EMEA/OD/031/08 Avian polyclonal IgY antibody against Pseudomonas aeruginosa, EMEA/OD/010/08 N-(2,4-Di-tert-butyl-5-hydroxyphenyl)-1,4-dihydro-4-oxoquinoline-3-carboxamide, EMEA/OD/009/09 Hypothiocyanite / lactoferrin, EMA/OD/040/10 Nafamostat mesilate, EMA/OD/032/11 Sinapultide, dipalmitoylphosphatidylcholine palmitoyl-oleoyl phosphatidylglycerol, sodium salt and palmitic acid, EMA/OD/037/11 Multilamellar microvesicle comprising phosphatidylcholine, sphingomyelin, phosphatidylethanolamine, phosphatidylserine, phospatidylinositol and cholesterol, EMA/OD/046/11 Cysteamine, EMA/OD/058/12 Alpha-1 proteinase inhibitor (for inhalation use), EMA/OD/005/13 Recombinant human CXCL8 mutant, EMA/OD/017/13 4,6,4'-trymethylangelicin, EMA/OD/096/13 Antisense oligonucleotide targeting the F508delta mutation of CFTR, EMA/OD/095/13 Nitric oxide, EMA/OD/159/13 Cysteamine, EMA/OD/156/13 11-(4-Dimethylamino-3-hydroxy-6-methyl-tetrahydro-pyran-2-yloxy)-2-ethyl-3,4,10-trihydroxy-3,5,6,8,10,12,14-heptamethyl-1-oxa-6-aza-cyclopentadecane-13,15-dione, EMA/OD/036/14 Nitric oxide, EMA/OD/013/14 Plasmid DNA encoding the human cystic fibrosis transmembrane conductance regulator gene complexed with a non-viral, cationic lipid based gene transfer agent, EMA/OD/002/14 1-(2,2-difluoro-1,3-benzodioxol-5-yl)-N-{1-[(2R)-2,3-dihydroxypropyl]-6-fluoro-2-(1-hydroxy-2-methylpropan-2-yl)-1Hindol-5-yl}cyclopropanecarboxamide, EMA/OD/131/14 4-[[(1S,4S)-5-[[4-[4-(Oxazol-2-yl)phenoxy]phenyl]methyl]-2,5-diazabicyclo[2.2.1]hept-2-yl]methyl]benzoic acid, EMA/OD/018/15 2-(7-ethoxy-4-(3-fluorophenyl)-1-oxophthalazin-2(1H)-yl)-N-methyl-N-(2-methylbenzo[d]oxazol-6-yl)acetamide, EMA/OD/319/14 Nitric oxide, EMA/OD/068/15 Fixed-dose combination of fosfomycin disodium and tobramycin, EMA/OD/061/15 Recombinant human acid ceramidase, EMA/OD/013/16 Sodium nitrite and ethylenediaminetetraacetic acid, EMA/OD/156/16 1-(2,2-difluoro-2H-1,3-benzodioxol-5-yl)-N-{1-[(2R)-2,3-dihydroxypropyl]-6-fluoro-2-(1-hydroxy-2-methylpropan-2-yl)-1H-indol-5-yl}cyclopropane-1-carboxamide and ivacaftor, EMA/OD/100/16 (6aR, 10aR)-3-(1',1'-dimethylheptyl)- delta-8-tetrahydro-cannabinol-9-carboxylic acid, EMA/OD/303/16 Phosphoinositide 3-kinase gamma peptide, EMA/OD/006/17 Tamoxifen citrate

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/107/04 3-[5-(2-fluoro-phenyl)-[1,2,4]oxadiazole-3-yl]-benzoic acid, EMEA/OD/054/05 Heparin sodium (inhalation use), EMEA/OD/072/05 Denufosol tetrasodium, EMEA/OD/118/05 Glutathione, EMEA/OD/024/08 Levofloxacin hemihydrate, EMA/OD/024/10 3-(6-(1-(2,2-difluorobenzo [d] [1,3] dioxol-5-yl)cyclopropanecarboxamido)-3-methylpyridin-2-yl)benzoic acid, EMA/OD/032/14 Lumacaftor/ivacaftor

2.2.24. - EMA/OD/089/17

Treatment of pulmonary arterial hypertension

Action: For discussion/adoption

Document(s) tabled:
Draft Summary report

Notes:
There have been 5 designations for this condition: Please see 2.2.22.

2.3. Revision of the COMP opinions

None

2.4. Amendment of existing orphan designations

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(s) tabled:
OMPD applications - appointment of coord. at the 11-13 July 2017 COMP meeting

2.7. Evaluation on-going

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

Action: For information

Notes:
See 7.8.1. Evaluation Ongoing.

3. Requests for protocol assistance with significant benefit question

3.1. Ongoing procedures

3.1.1. -

Treatment of acute hepatic porphyria

Action: For adoption

3.1.2. -

Treatment of Prader-Willi syndrome

Action: For adoption

3.1.3. -

Treatment in solid organ transplantation

Action: For adoption

3.1.4. -

Treatment of haemophilia A

Action: For adoption

3.2. Finalised letters

3.2.1. -

Treatment of myasthenia gravis

Action: For information

3.2.2. -

Treatment of mercury toxicity

Action: For information

3.2.3. -

Treatment of plasminogen deficiency

Action: For information

3.2.4. -

Treatment of graft-versus-host disease

Action: For information

3.3. New requests

3.3.1. -

Treatment of paroxysmal nocturnal haemoglobinuria

Action: For information

3.3.2. -

Treatment of congenital factor VII deficiency

Action: For information

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

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

4.1.1. Soliris - eculizumab – Type II variation - EMEA/OD/062/14, EU/3/14/1304, EMEA/H/C/000791/II/0090

Alexion Europe SAS - France; Treatment of myasthenia gravis

CHMP rapporteur: Jorge Camarero Jiménez

Action: For information

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

Notes:
Status of the procedure at the CHMP: Opinion adopted in June 2017. The COMP adopted the opinion by written procedure following its June meeting.

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

4.2.1. Adcetris - Brentuximab vedotin – Type II variation - EMA/OD/100/11, EU/3/11/939, EMEA/H/C/002455/II/0048

Takeda Pharma A/S - Denmark; Treatment of cutaneous T-cell lymphoma

CHMP rapporteur: Paula Boudewina van Hennik; CHMP co-rapporteur: Jan Mueller-Berghaus

Action: For information

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

4.2.2. - midostaurin – EMEA/H/C/004095

Novartis Europharm Ltd;

a) Treatment of mastocytosis, EMA/OD/016/10, EU/3/10/765

b) Treatment of acute myeloid leukaemia, EMEA/OD/028/04, EU/3/04/214

Action: For adoption, Oral explanation to be held on 11 July 2017 at 15:30

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

4.2.3. - ciclosporin – EMEA/OD/106/05, EU/3/06/360, EMEA/H/C/004411

Santen Oy; Treatment of vernal keratoconjunctivitis

Action: For adoption

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

4.2.4. - telotristat ethyl – EMEA/OD/047/09, EU/3/09/661, EMEA/H/C/003937

Ipsen Pharma; Treatment of carcinoid syndrome

Action: For adoption

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

4.2.5. - lutetium 177Lu dotatate – EMEA/OD/093/07, EU/3/07/523, EMEA/H/C/004123

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

Action: For adoption

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

4.2.6. – avelumab – EMA/OD/150/15, EU/3/15/1590, EMEA/H/C/004338

Merck Serono Europe Limited; Treatment of Merkel cell carcinoma

Action: For discussion

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

4.3. Appeal

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

GMP-Orphan SA; Treatment of Wilson's disease

Action: For adoption, Oral explanation to be held on 11 July 2017 at 14:30

Document(s) tabled:
Revised draft Summary report
Sponsor's grounds for appeal

Notes:
Status of the procedure at the CHMP: Opinion adopted in April 2017. Appeal of the COMP negative opinion adopted on 23 May 2017.

4.4. On-going procedures

Action: For information

Document(s) tabled:
Review of orphan designation for OMP for MA - On-going procedures

4.5. Public Summary of Opinions

Action: For information

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

5.1. After adoption of CHMP opinion

None

5.2. Prior to adoption of CHMP opinion

5.2.1. Blincyto (blinatumomab) - Type II variation – EMEA/OD/029/09, EU/3/09/650, EMEA/H/C/003731/II/0018

Amgen Europe BV - The Netherlands; Treatment of acute lymphoblastic leukaemia

CHMP rapporteur: Alexandre Moreau

Action: For information

Documents tabled:
Draft report on review of OMPD

5.3. Appeal

None

5.4. On-going procedures

Action: For information

Document(s) tabled:
Review of orphan designation for OMP for MA extension - On-going procedures

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

None

7. Organisational, regulatory and methodological matters

7.1. Mandate and organisation of the COMP

7.1.1. COMP Strategic Review & Learning meeting, 19-21 September 2017, Lisbon, Portugal

Action: For information

7.1.2. Protocol Assistance Working Group

Proposed meeting time on 11 July 2017 at 13:00

Document(s) tabled:
PAWG draft minutes for June 2017 meeting

7.1.3. Prevalence Working Group

Proposed meeting time on 12 July 2017 at 08:30

Document(s) tabled:
Prevalence WG draft agenda for July 2017 meeting

7.2. Coordination with EMA Scientific Committees or CMDh-v

7.2.1. Recommendations on eligibility to PRIME – report from CHMP

Action: For information

Document(s) tabled:
PRIME eligibility requests - list of adopted outcomes June 2017

7.2.2. PDCO/COMP Working Group

Proposed meeting time on 12 July 2017 at 13:00

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

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

PCWP/HCPWP joint meeting - 15 March 2017

Action: For information

Document(s) tabled:
Minutes PCWP/HCPWP joint meeting – 15 March (EMA/182189/2017)

7.4. Cooperation within the EU regulatory network

7.4.1. European Commission

None

7.5. Cooperation with International Regulators

7.5.1. Food and Drug Administration (FDA)

Action: For information

Document(s) tabled:

Draft Agenda of EMA/FDA teleconference on Orphan Medicines June 20, 2017

Notes:
Monthly teleconference

7.5.2. Japanese Pharmaceuticals and Medical Devices Agency (PMDA)

Action: For information

Notes:
Ad hoc basis meeting

7.5.3. The Therapeutic Goods Administration (TGA), Australia

Action: For information

Notes:
Ad hoc basis meeting

7.5.4. Health Canada

Action: For information

Notes:
Ad hoc basis meeting

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

None

7.7. COMP work plan

Action: For information

Document(s) tabled:
COMP Work Plan 2017

7.8. Planning and reporting

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

Action: For information

7.8.2. Overview of orphan marketing authorisations/applications

Action: For information

8. Any other business

8.1. Publication of review of orphan criteria report

Action: For information/discussion

9. Explanatory notes

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

Abbreviations / Acronyms

CHMP: Committee for Medicinal Product for Human Use

COMP: Committee for Orphan Medicinal Products

EC: European Commission

OD: Orphan Designation

PA: Protocol Assistance

PDCO: Paediatric Committee

PRAC: Pharmacovigilance and Risk Assessment Committee

SA: Scientific Advice

SAWP: Scientific Advice Working Party

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

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

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

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

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

Sponsor

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

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

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

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