20 April 2016
EMA/COMP/225244/2016
Procedure Management and Committees Support Division

Committee for Orphan Medicinal Products (COMP)

Draft agenda for the meeting on 19-21 April 2016

Chair: Bruno Sepodes – Vice-Chair: Lesley Greene

19 April 2016, 09:00-18:30, room 2F

20 April 2016, 09:00-18:30, room 2F

21 April 2016, 08:30-12:30, room 2F

Health and safety information

In accordance with the Agency's health and safety policy, delegates are to be briefed on health, safety and emergency information and procedures prior to the start of the meeting.

Disclaimers

Some of the information contained in this agenda is considered commercially confidential or sensitive and therefore not disclosed. With regard to intended therapeutic indications or procedure scopes listed against products, it must be noted that these may not reflect the full wording proposed by applicants and may also vary during the course of the review. Additional details on some of these procedures will be published in the COMP meeting reports once the procedures are finalised.

Of note, this agenda is a working document primarily designed for COMP members and the work the Committee undertakes.

Note on access to documents

Some documents mentioned in the agenda cannot be released at present following a request for access to documents within the framework of Regulation (EC) No 1049/2001 as they are subject to on-going procedures for which a final decision has not yet been adopted. They will become public when adopted or considered public according to the principles stated in the Agency policy on access to documents (EMA/127362/2006).

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1. Introduction

1.1. Welcome and declarations of interest of members and experts

Pre-meeting list of participants and restrictions in relation to declarations of interests applicable to the items of the agenda for the COMP plenary session to be held 19-21 April 2016. See April 2016 COMP minutes (to be published post May 2016 COMP meeting).

1.2. Adoption of agenda

COMP agenda for 19-21 April 2016.

1.3. Adoption of the minutes

COMP minutes for 21-23 March 2016.

2. Applications for orphan medicinal product designation

2.1. For opinion

2.1.1. - EMA/OD/256/15

Treatment of inclusion body myositis

Action: For adoption, Oral explanation to be held on 19 April 2016 at time 11:00

Documents tabled:
Draft Summary report with response to LoQs

Notes:
There is currently 1 designation for this condition: EMA/OD/046/12 recombinant human monoclonal antibody against activin receptor type IIB

2.1.2. - EMA/OD/220/15

Treatment of peripheral T-cell lymphoma

Action: For adoption, Oral explanation to be held on 19 April 2016 at time 17:00

Documents tabled:
Draft Summary report with response to LoQs

Notes:
There are currently 8 designations for this condition: EMA/OD/047/12 Recombinant anti-CD3-bi-single-chain-Fv-diphtheria toxin fusion protein, EMA/OD/104/12 Alisertib, EMA/OD/103/12 Belinostat, EMEA/OD/056/05 (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/100/06 Pralatrexate, EMEA/OD/096/06 Zanolimumab, EMA/OD/112/10 Darinaparsin, EMA/OD/104/11 Mogamulizumab

2.1.3. - EMA/OD/146/15

Treatment of congenital coronary artery malformation

Action: For information

Documents tabled:
Withdrawal request of 12 April 2016

2.1.4. - EMA/OD/246/15

Treatment of paroxysmal nocturnal hemoglobinuria

Action: For adoption, Oral explanation to be held on 19 April 2016 at time 09:30

Documents tabled:
Draft Summary report with response to LoQs

2.1.5. - EMA/OD/258/15

Treatment of glioma

Action: For adoption, Oral explanation to be held on 20 April 2016 at time 17:00

Documents tabled:
Draft Summary report with response to LoQs

Notes:
There are currently 41 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 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, EMA/OD/206/15 N-(4-Methoxyphenyl)-N,2,6-trimethylfuro[2,3-d]pyrimidin-4-amine, EMA/OD/222/15 Delta-9-tetrahydrocannabinol and cannabidiol from extracts of the Cannabis sativa L. plant

Designations withdrawn: EMEA/OD/067/01 Carmustine (solution for intratumoral injection), EMEA/OD/074/01 Human transferrin conjugated to mutant diptheria toxin, EMEA/OD/067/03 Cilengitide, EMEA/OD/050/06 Iodine (131I) anti-tenascin monoclonal antibody 81C6, EMEA/OD/112/08 Talampanel, EMEA/OD/004/09 4,6,8-trihydroxy-10-(3,7,11-trimethyldodeca-2,6,10-trienyl)-5,10-dihydrodibenzo[b,e][1,4] diazepin-11-one, EMA/OD/031/10 Glutathione-pegylated liposomal doxorubicin hydrochloride, EMA/OD/049/12 Humanised monoclonal antibody against epidermal growth factor receptor, EMA/OD/113/15 Dronabinol and cannabidiol

2.2. For discussion / preparation for an opinion

2.2.1. - EMA/OD/245/15

Treatment of biliary tract cancer

Action: For adoption

Documents tabled:
Draft Summary report

Notes:
There is currently 1 designation for this condition: EMA/OD/305/14 5,10,15,20-tetrakis(2,6-difluoro-3-N-methylsulfamoylphenyl)bacteriochlorin

2.2.2. - EMA/OD/023/16

Treatment of acromegaly

Action: For adoption

Documents tabled:
Draft Summary report

Notes:
There are currently 4 designations for this condition: EMEA/OD/010/09 Octreotide chloride (lipid depot solution), EMEA/OD/051/09 Pasireotide, EMA/OD/107/12 Cyclo(-gamma-aminobutyryl-L-phenylalanyl-L-tryptophanyl-D-tryptophanyl-L-lysyl-L-threonyl-L phenylalanyl-N-3-carboxypropyl)-glycine amide, acetate salt, EMA/OD/042/13 Octreotide acetate (oral use)

Designation withdrawn: EMA/OD/108/11 Recombinant protein consisting of modified human growth hormone releasing hormone and the translocation and endopeptidase domains of botulinum toxin serotype D

2.2.3. - EMA/OD/016/16

Treatment of diffuse large B-cell lymphoma

Action: For adoption

Documents tabled:
Draft Summary report

Notes:
There are currently 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/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'-deoxyguanosordinator

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

2.2.4. - EMA/OD/208/15

Treatment of retinitis pigmentosa

Action: For adoption

Documents tabled:
Draft Summary report

Notes:
There are currently 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/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/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/067/13 Unoprostone isopropyl, 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

Designations withdrawn: EMEA/OD/075/07 Recombinant human rod-derived cone viability factor, EMEA/OD/106/07 Allogeneic human umbilical cord tissue-derived cells

2.2.5. - EMA/OD/019/16

Treatment of Fanconi anaemia

Action: For adoption

Documents tabled:
Draft Summary report

2.2.6. - EMA/OD/020/16

Treatment of severe combined immunodeficiency

Action: For adoption

Documents tabled:
Draft Summary report

Notes:
There is currently 1 designation for this condition: EMEA/OD/060/00 Retroviral gamma c cDNA containing vector

2.2.7. - EMA/OD/021/16

Treatment of Wiskott-Aldrich syndrome

Action: For adoption

Documents tabled:
Draft Summary report

Notes:
There are currently 2 designations for this condition: EMA/OD/014/12 Autologous CD34+ cells transfected with lentiviral vector containing the Wiskott-Aldrich syndrome protein gene, EMA/OD/104/13 Autologous CD34+ cells transduced with a lentiviral vector containing the human Wiskott-Aldrich syndrome gene

2.2.8. - EMA/OD/018/16

Treatment of beta thalassaemia intermedia and major

Action: For adoption

Documents tabled:
Draft Summary report

Notes:
There is currently 1 designation for this condition: EMA/OD/092/15 Synthetic hepcidin

2.2.9. - EMA/OD/024/16

Treatment of beta thalassemia intermedia and major

Action: For adoption

Documents tabled:
Draft Summary report

2.2.10. - EMA/OD/022/16

Treatment of acute liver failure

Action: For adoption

Documents tabled:
Draft Summary report

Notes:
There are currently 6 designations for this condition: EMEA/OD/037/05 Human heterologous liver cells (for infusion), EMEA/OD/085/08 Recombinant human hepatocarcinoma-intestine-pancreas / pancreatic associated protein, EMA/OD/030/11 Cardiotrophin-1, EMA/OD/105/11 Ornithine phenylacetate, EMA/OD/153/11 Heterologous human adult liver-derived stem cells, EMA/OD/032/13 Immortalised human C3A hepatoblastoma cells

2.2.11. - EMA/OD/008/16

Treatment of acute lymphoblastic leukaemia

Action: For adoption

Documents tabled:
Draft Summary report

Notes:
There are currently 20 designations for this condition: EMEA/OD/046/01 2-chloro-9-[2-deoxy-2-fluoro-ß-D-arabinofuranosyl]adenine, EMEA/OD/070/06 Forodesine hydrochloride, EMEA/OD/015/05 Nelarabine, EMEA/OD/074/05 Dasatinib, EMEA/OD/033/06 L-asparaginase encapsulated in erythrocytes, EMEA/OD/065/07 Mercaptopurine (oral liquid), EMEA/OD/064/07 Methotrexate (oral liquid), EMEA/OD/002/08 Vincristine sulphate liposomes, EMEA/OD/032/08 Pegylated L-asparaginase, 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, EMA/OD/107/15 Autologous T cells transduced with retroviral vector encoding an anti-CD19 CD28/CD3-zeta chimeric antigen receptor, EMA/OD/090/15 Allogeneic umbilical cord blood cells treated ex vivo with 16,16-dimethyl prostaglandin E2

Designations withdrawn: EMEA/OD/022/03 Aplidine, EMEA/OD/063/04 L-Asparaginase, EMEA/OD/038/05 Imatinib mesilate, EMEA/OD/067/08 Allogeneic ex vivo expanded umbilical cord blood cells

2.2.12. - EMA/OD/009/16

Treatment of glioma

Action: For adoption

Documents tabled:
Draft Summary report

Notes:
There are currently 41 designations for this condition: Please see 2.1.5.

2.2.13. - EMA/OD/010/16

Treatment of glioma

Action: For adoption

Documents tabled:
Draft Summary report

Notes:
There are currently 41 designations for this condition: Please see 2.1.5.

2.2.14. - EMA/OD/011/16

Treatment of amyotrophic lateral sclerosis

Action: For adoption

Documents tabled:
Draft Summary report

Notes:
There are currently 14 designations for this condition: EMEA/OD/053/06 Arimoclomol, EMEA/OD/102/07 Filgrastim, EMEA/OD/096/08 (6R)-4,5,6,7-tetrahydro-N6-propyl-2,6-benzothiazole-diamine dihydrochloride monohydrate, EMEA/OD/108/09 Recombinant human vascular endothelial growth factor, EMA/OD/043/11 Smilagenin, EMA/OD/106/11 S[+] apomorphine, EMA/OD/138/11 6-ethynyl-1-(pentan-3-yl)-1H-imidazo[4,5-b]pyrazin-2(3H)-one, EMA/OD/011/13 Autologous bone marrow-derived mesenchymal stromal cells secreting neurotrophic factors, EMA/OD/023/13 Sodium chlorite, EMA/OD/044/13 Allogeneic

motor neuron progenitor cells derived from human embryonic stem cells, EMA/OD/184/14 Edaravone, EMA/OD/283/14 Enoxacin, EMA/OD/032/15 Edaravone, EMA/OD/051/15 Hydrocinnamate-[Orn-Pro-dCha-Trp-Arg]acetate

Designations withdrawn: EMEA/OD/029/00 Xaliproden hydrochloride, EMEA/OD/030/06 Cholest-4-en-3-one, oxime, EMEA/OD/125/07 Sarsasapogenin, EMEA/OD/012/09 Talampanel, EMA/OD/060/10 Recombinant humanised monoclonal antibody to human Nogo-A protein of the IgG1/kappa class

2.2.15. - EMA/OD/014/16

Treatment of neuromyelitis optica

Action: For adoption

Documents tabled:
Draft Summary report

Notes:
There is currently 1 designation for this condition: EMA/OD/087/13 Eculizumab

2.2.16. - EMA/OD/004/16

Treatment of eosinophilic oesophagitis

Action: For adoption

Documents tabled:
Draft Summary report

Notes:
There are currently 2 designations for this condition: EMA/OD/078/13 Budesonide, EMA/OD/118/13 Human monoclonal antibody against human interleukin 13

2.2.17. - EMA/OD/007/16

Treatment of neonatal sepsis

Action: For adoption

Documents tabled:
Draft Summary report

2.2.18. - EMA/OD/001/16

Treatment of necrotising enterocolitis

Action: For adoption

Documents tabled:
Draft Summary report

2.2.19. - EMA/OD/005/16

Treatment of acute respiratory distress syndrome

Action: For adoption

Documents tabled:
Draft Summary report

Notes:
There are currently 2 designations for this condition: EMEA/OD/099/06 Drotrecogin alfa (activated), EMA/OD/110/14 Imatinib

2.2.20. - EMA/OD/003/16

Treatment of interstitial cystitis

Action: For adoption

Documents tabled:
Draft Summary report

Notes:
There is currently 1 designation for this condition: EMA/OD/179/14 Pentosan polysulfate sodium

2.2.21. - EMA/OD/025/16

Treatment of homocystinuria

Action: For adoption

Documents tabled:
Draft Summary report

Notes:
There is currently 1 designation for this condition: EMEA/OD/003/00 Betaine anhydrous

2.2.22. - EMA/OD/002/16

Treatment of retinitis pigmentosa caused by mutations in the RPGR gene

Action: For adoption

Documents tabled:
Draft Summary report

2.2.23. - EMA/OD/017/16

Treatment of graft versus host disease

Action: For adoption

Documents tabled:
Draft Summary report

2.2.24. - EMA/OD/015/16

Treatment of small cell lung cancer

Action: For adoption

Documents tabled:
Draft Summary report

Notes:
There are currently 2 designations for this condition: EMEA/OD/113/07 Amrubicin hydrochloride, EMA/OD/086/14 2-(2-methyl-5-nitro-1H-imidazol-1-yl)ethylsulfamide

Designations withdrawn: EMEA/OD/056/04 Sabarubicin, EMEA/OD/055/07 Picoplatin, EMA/OD/056/10 Maytansinoid-conjugated humanised monoclonal antibody against CD56

2.2.25. - EMA/OD/013/16

Treatment of cystic fibrosis

Action: For adoption

Documents tabled:
Draft Summary report

Notes:
There are currently 36 designations for this condition: EMEA/OD/011/03 Recombinant dog gastric lipase, EMEA/OD/032/00 L-Lysine-N-Acetyl-L-Cysteinate, EMEA/OD/038/02 Duramycin, EMEA/OD/039/04 Dexamethasone sodium phosphate encapsulated in human erythrocytes, EMEA/OD/053/04 Alpha-1 antitrypsin (inhalation use), EMEA/OD/107/04 3-[5-(2-fluoro-phenyl)-[1,2,4]oxadiazole-3-yl]-benzoic acid, EMEA/OD/062/05 Mannitolum, EMEA/OD/072/05 Denufosol tetrasodium, EMEA/OD/001/06 Heparin sodium, EMEA/OD/037/09 Ciprofloxacin (liposomal), EMEA/OD/092/06 Ciprofloxacin (inhalation use), EMEA/OD/104/06 Alginate oligosaccharide (G-block) fragment, EMEA/OD/041/07 Alpha1-proteinase inhibitor (inhalation use), EMEA/OD/031/08 Avian polyclonal IgY antibody against Pseudomonas aeruginosa, EMEA/OD/010/08 N-(2,4-Di-tert-butyl-5-hydroxyphenyl)-1,4-dihydro-4-oxoquinoline-3-carboxamide, EMEA/OD/009/09 Hypothiocyanite / lactoferrin, EMA/OD/040/10 Nafamostat mesilate, EMA/OD/024/10 3-(6-(1-(2,2-difluorobenzo [d] [1,3] dioxol-5-yl)cyclopropanecarboxamido)-3-methylpyridin-2-yl)benzoic acid, EMA/OD/032/11 Sinapultide, dipalmitoylphosphatidylcholine palmitoyl-oleoyl phosphatidylglycerol, sodium salt and palmitic acid, EMA/OD/037/11 Multilamellar microvesicle comprising phosphatidylcholine, sphingomyelin, phosphatidylethanolamine, phosphatidylserine, phospatidylinositol and cholesterol, EMA/OD/046/11 Cysteamine, EMA/OD/058/12 Alpha-1 proteinase inhibitor (for inhalation use), EMA/OD/005/13 Recombinant human CXCL8 mutant, EMA/OD/017/13 4,6,4'-trymethylangelicin, EMA/OD/096/13 Antisense oligonucleotide targeting the F508delta mutation of CFTR, EMA/OD/095/13 Nitric oxide, EMA/OD/159/13 Cysteamine, EMA/OD/156/13 11-(4-Dimethylamino-3-hydroxy-6-methyl-tetrahydro-pyran-2-yloxy)-2-ethyl-3,4,10-trihydroxy-3,5,6,8,10,12,14-heptamethyl-1-oxa-6-aza-cyclopentadecane-13,15-dione, EMA/OD/036/14 Nitric oxide, EMA/OD/013/14 Plasmid DNA encoding the human cystic fibrosis transmembrane conductance regulator gene complexed with a non-viral, cationic lipid based gene transfer agent, EMA/OD/002/14 1-(2,2-difluoro-1,3-benzodioxol-5-yl)-N-{1-[(2R)-2,3-dihydroxypropyl]-6-fluoro-2-(1-hydroxy-2-methylpropan-2-yl)-1Hindol-5-yl}cyclopropanecarboxamide, EMA/OD/131/14 4-[[(1S,4S)-5-[[4-[4-(Oxazol-2-yl)phenoxy]phenyl]methyl]-2,5-diazabicyclo[2.2.1]hept-2-yl]methyl]benzoic acid, EMA/OD/018/15 2-(7-ethoxy-4-(3-fluorophenyl)-1-oxophthalazin-2(1H)-yl)-N-methyl-N-(2-methylbenzo[d]oxazol-6-yl)acetamide, EMA/OD/319/14 Nitric

oxide, EMA/OD/068/15 Fixed-dose combination of fosfomycin disodium and tobramycin, EMA/OD/061/15 Recombinant human acid ceramidase

Designations withdrawn: EMEA/OD/009/02 Carbamic acid/[[4-[[3-[[4-[1-(4-hydroxyphenyl)-1-methyl-ethyl]phenoxy]methyl]phenyl]methoxy]-phenyl]iminomethyl]-,ethyl ester, EMEA/OD/064/00 8-cyclopentyl-1, 3-dipropylxanthine, EMEA/OD/018/03 Engineered protein inhibitor of human neutrophil elastase, EMEA/OD/075/02 Amiloride hydrochloride dihydrate, EMEA/OD/023/04 Recombinant human bile salt-stimulated lipase, EMEA/OD/054/05 Heparin sodium (inhalation use), EMEA/OD/118/05 Glutathione, EMEA/OD/024/08 Levofloxacin hemihydrate, EMA/OD/032/14 Lumacaftor/ivacaftor

2.2.26. - EMA/OD/027/16

Treatment of adrenoleukodystrophy

Action: For adoption

Documents tabled:
Draft Summary report

Notes:
There are currently 2 designations for this condition: EMA/OD/009/12 Autologous haematopoietic stem cells transduced with lentiviral vector Lenti-D encoding the human ABCD1 cDNA, EMA/OD/170/13 Pioglitazone

2.2.27. - EMA/OD/026/16

Treatment of Langerhans cell histiocytosis

Action: For adoption

Documents tabled:
Draft Summary report

2.3. Amendment of the COMP opinions

2.3.1. (S)-ethyl 2-amino-3-(4-(2-amino-6((R)-1-(4-chloro-2-(3-methyl-1H-pyrazol-1-yl)phenyl)-2,2,2-trifluoroethoxy)pyrimidin-4-yl)phenyl)propanoate – EMA/OD/047/09, EU/3/09/66

Ipsen Pharma - France; Treatment of carcinoid tumours

Action: For adoption

Document tabled:
Amended draft Summary report

2.4. COMP opinions adopted via written procedure following previous meeting

None

2.5. Appeal

None

2.6. Nominations

2.6.1. New applications for orphan medicinal product designation - Appointment of COMP coordinators

Action: For adoption

Document tabled:
OMPD applications - appointment of coord. at the 19-21 April 2016 COMP meeting

2.7. Evaluation on-going

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

Action: For information

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

3. Requests for protocol assistance with significant benefit question

3.1. Ongoing procedures

3.1.1. -

Treatment of primary sclerosing cholangitis

Action: For adoption

3.1.2. -

Treatment of beta thalassaemia intermedia and major

Action: For adoption/discussion

3.2. Finalised letters

3.2.1. -

Treatment of hyperargininaemia

Action: For information

3.2.2. -

Treatment of argininosuccinic aciduria

Action: For information

3.2.3. -

Treatment of haemophilia A

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. Darzalex – daratumumab - EMA/OD/038/13, EU/3/13/1153 , EMEA/H/C/004077

Janssen-Cilag International N.V.; Treatment of plasma cell myeloma

Action: For adoption, Oral explanation to be held on 19 April 2016 at 12:00

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

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

4.1.2. Empliciti - elotuzumab - EMA/OD/061/12, EU/3/12/1037, EMEA/H/C/003967

Bristol-Myers Squibb; Treatment of multiple myeloma

Action: For information

Document(s) tabled:
Report on review of OMPD

Notes:
Status of the procedure at the CHMP: Opinion adopted in January 2016.
After appeal of the sponsor, the COMP adopted a final negative opinion by written procedure following its March meeting.

4.1.3. Galafold - migalastat – EMEA/OD/105/05, EU/3/06/368, EMEA/H/C/004059

Amicus Therapeutics UK Ltd; Treatment of Fabry disease

Action: For information

Document(s) tabled:
Report on review of OMPD

Notes:
Status of the procedure at the CHMP: Opinion adopted at March CHMP.
The COMP opinion was adopted by written procedure after its March meeting.

4.1.4. Strimvelis - autologous cd34+ enriched cell fraction that contains cd34+ cells transduced with retroviral vector that encodes for the human ada cdna sequence EMEA/OD/053/05, EU/3/05/313, EMEA/H/C/003854

GlaxoSmithKline Trading Services; Treatment of severe combined immunodeficiency (SCID) due to adenosine deaminase (ADA) deficiency

Action: For information

Document(s) tabled:
Report on review of OMPD

Notes:
Status of the procedure at the CHMP: Opinion adopted at March CHMP.
The COMP opinion was adopted by written procedure after its March meeting.

4.1.5. Dropcys (CYSTIRANE) – mercaptamine – EMA/OD/106/14, EU/3/14/1341, EMEA/H/C/004038

Lucane Pharma; Treatment of cystinosis

Action: For information

Notes:
Status of the procedure at the CHMP: Negative opinion on the re-examination adopted at March CHMP.

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

4.2.1. - chenodeoxycholic acid – EMA/OD/196/14, EU/3/14/1406, EMEA/H/C/004061

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

Action: For information

4.2.2. – obinutuzumab - Type II variation - EMA/OD/013/15, EU/3/15/1504, EMEA/H/C/002799/II/0007

Roche Registration Limited; Treatment of follicular lymphoma

Action: For discussion

Documents tabled:
Draft report on review of OMPD

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

Baxter Innovations GmbH; Treatment of pancreatic cancer

Action: For information

4.2.4. – ixazomib – EMA/OD/110/12, EU/3/12/1060, EMEA/H/C/003844

Takeda Pharma A/S; Treatment of systemic light chain amyloidosis

Action: For information

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

NPS Pharma Holdings Limited; Treatment of hypoparathyroidism

Action: For information

4.2.6. - sirolimus – EMA/OD/021/11, EU/3/11/898, EMEA/H/C/003978

Santen Oy; Treatment of chronic non-infectious uveitis

Action: For information

4.3. On-going procedures

4.3.1. List of on-going procedures

Action: For information

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

None

6. Organisational, regulatory and methodological matters

6.1. Mandate and organisation of the COMP

6.1.1. Significant Benefit Working Group

Proposed meeting time on 21 April 2016 at time 08:30, room 2B

6.1.2. Protocol Assistance Working Group

Proposed meeting time on 20 April 2016 at time 12:00, room 2H

6.2. Coordination with EMA Scientific Committees or CMDh-v

6.2.1. CHMP guideline on Conditional Marketing Authorisation

Update to the COMP on final guideline

Action: For information

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

6.3.1. SAWP/COMP joint membership

Renewal of Armando Magrelli as SAWP/COMP member for a second term

Action: For adoption

6.4. Cooperation within the EU regulatory network

6.4.1. European Commission

Comments received during the public consultation on the Commission Notice on the application of Articles 3,5 and 7 of Regulation (EC) NO 141/2000 on Orphan Medicinal Products

Action: For discussion

Document(s) tabled:
Annex 1 - Notice on orphan medicinal products
Background document for the COMP meeting on April 2016

6.5. Cooperation with International Regulators

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

None

6.8. Planning and reporting

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

Action: For information

6.8.2. Overview of orphan marketing authorisations/applications

Action: For information

7. Any other business

7.1. Request for clarification of the condition / indication

The EMA received on 3 March 2016 from the sponsor a request for clarification of the condition/indication for orphan drug designation 'Chimeric monoclonal antibody against claudin-18 splice variant 2 for the treatment of gastric cancer' (EU/3/10/803)

Action: For adoption

Document(s) tabled:
Sponsor's letter