6 July 2015
EMA/COMP/453252/2015
Procedure Management and Committees Support Division

Committee for Orphan Medicinal Products (COMP)

Draft agenda for the meeting on 14-16 July 2015

Chair: Bruno Sepodes – Vice-Chair: Lesley Greene

14 July 2015, 09:00-18:30, room 3E

15 July 2015, 08:30-18:30, room 3E

16 July 2015, 08:30-13:00, room 3E

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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Table of contents

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 14-16 July 2015. See July 2015 COMP minutes (to be published post September 2015 COMP meeting).

1.2. Adoption of agenda

COMP agenda for 14-16 July 2015.

1.3. Adoption of the minutes

COMP minutes for 16-18 June 2015.

2. Applications for orphan medicinal product designation

2.1. For opinion

2.1.1. - EMA/OD/050/15

Treatment of short bowel syndrome

Action: For adoption, Oral explanation to be held on 14 July 2015 at time 09:30

Documents tabled:

Draft Summary report with response to LoQs

Notes:
There have been 2 designations for this condition: EMEA/OD/045/01 [gly2]-recombinant human glucagon-like peptide, EMA/OD/080/14 Oxalobacter formigenes strain HC-1

2.1.2. - EMA/OD/038/15

Treatment of plasma cell myeloma

Action: For adoption, Oral explanation to be held on 14 July 2015 at time 11:00

Documents tabled:

Draft Summary report with response to LoQs

Reader's guidance

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

2.1.3. - EMA/OD/039/15

Treatment of cystic fibrosis

Action: For adoption, Oral explanation to be held on 14 July 2015 at time 12:00

Documents tabled:

Draft Summary report with response to LoQs

Reader's guidance

Notes:
There have been 36 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, EMA/OD/024/10 3-(6-(1-(2,2-difluorobenzo [d] [1,3] dioxol-5-yl)cyclopropanecarboxamido)-3-methylpyridin-2-yl)benzoic acid, EMEA/OD/039/04 Dexamethasone sodium phosphate encapsulated in human erythrocytes, EMEA/OD/053/04 Alpha-1 antitrypsin (inhalation use), 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/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/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/032/14 Lumacaftor/ivacaftor, 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
Designations withdrawn: EMEA/OD/064/00 8-cyclopentyl-1, 3-dipropylxanthine, 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/018/03 Engineered protein inhibitor of human neutrophil elastase, EMEA/OD/075/02 Amiloride hydrochloride dihydrate, EMEA/OD/118/05 Glutathione, EMEA/OD/054/05 Heparin sodium (inhalation use), EMEA/OD/024/08 Levofloxacin hemihydrate

2.1.4. - EMA/OD/049/15

Treatment of uveal melanoma

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

Documents tabled:

Draft Summary report with response to LoQs

2.1.5. - EMA/OD/056/15

Treatment of Rett Syndrome

Action: For adoption, Oral explanation to be held on 15 July 2015 at time 09:30

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

Notes:
There have been 2 designations for this condition: EMEA/OD/001/09 Desipramine chlorhydrate, EMA/OD/163/13 3-Chloro-4-fluorophenyl-[4-fluoro-4-{[(5-methylpyrimidin-2-ylmethyl) amino]methyl}piperidin-1-yl]methanone

2.1.6. - EMA/OD/053/15

Treatment of osteogenesis imperfecta and osteogenesis imperfecta-related disorders

Action: For adoption, Oral explanation to be held on 15 July 2015 at time 11:00

Documents tabled:

Draft Summary report with response to LoQs

2.2. For discussion / preparation for an opinion

2.2.1. - EMA/OD/085/15

Treatment of mitochondrial encephalomyopathy, lactic acidosis, and stroke-like episodes

Action: For adoption

Documents tabled:
Draft Summary report

2.2.2. - EMA/OD/087/15

Treatment of hepatocellular carcinoma

Action: For adoption

Documents tabled:
Draft Summary report
COMP coordinator's comments

Notes:
There have been 17 designations for this condition: EMEA/OD/015/02 Thymalfasin, EMEA/OD/087/04 Pegylated arginine deiminase, EMEA/OD/048/04 Doxorubicine polyisohexylcyanoacrylate nanoparticles, EMEA/OD/018/05 Nemorubicin hydrochloride, EMEA/OD/109/05 Sorafenib tosylate, EMEA/OD/090/07 N-[4-(3-amino-1H-indazol-4 yl)phenyl]-N'-(2-fluoro-5-methylphenyl) urea, EMEA/OD/070/09 NGR-human tumour necrosis factor, EMEA/OD/076/09 Vaccinia GM-CSF/TK-deactivated virus, EMA/OD/065/10 (S)-10-[(dimethylamino)methyl]-4-ethyl-9-hydroxy-4-O-[alpha-(2", 4", 5", 7"-tetranitro-9"-fluorenylideneaminooxy)propionyl]-1H-pyrano[3', 4', 6', 7']indolizino[1,2-beta]-quinoline-3, 14-(4H, 12H)-dione, hydrochloride, EMA/OD/096/10 Doxorubicin hydrochloride (in heat-sensitive liposomes), EMA/OD/170/10 Sulfonated monophosphorylated mannose oligosaccharide, EMA/OD/003/11 Peretinoin, EMA/OD/045/11 Resminostat, EMA/OD/031/12 Ramucirumab, EMA/OD/159/12 4-[2-(6-methylpyridin-2-yl)-5,6-dihydro-4H-pyrrolo[1,2-

b]pyrazol-3-yl]-quinoline-6-carboxamide monohydrate, EMA/OD/115/13 Tivantinib, EMA/OD/160/14 Diaspirin cross-linked haemoglobin

Designations withdrawn: EMEA/OD/013/01 Seocalcitol, EMEA/OD/026/02 Doxorubicin carbon/iron magnetically targeted microparticles, EMEA/OD/032/03 Nolatrexed, EMEA/OD/046/07 4-[3,5-bis(trimethylsilyl)benzamido] benzoic acid, EMA/OD/075/11 Brivanib alaninate

2.2.3. - EMA/OD/084/15

Treatment of diffuse large B-cell lymphoma

Action: For adoption

Documents tabled:

Draft Summary report

COMP coordinator's comments

Notes:
There have been 3 designations for this condition: EMEA/OD/097/06 Enzastaurin hydrochloride, EMA/OD/071/14 (Z)-3-(3-(3,5-bis(trifluoromethyl)phenyl)-1H-1,2,4-triazol-1-yl)-N'-(pyrazin-2-yl)acrylohydrazide, EMA/OD/171/14 Autologous T cells transduced with retroviral vector encoding an anti-CD19 CD28/CD3 zeta chimeric antigen receptor

2.2.4. - EMA/OD/074/15

Treatment of X-linked myotubular myopathy

Action: For adoption

Documents tabled:

Draft Summary report

2.2.5. - EMA/OD/076/15

Treatment of mucopolysaccharidosis type II (Hunter's syndrome)

Action: For adoption

Documents tabled:

Draft Summary report

Notes:
There has been 1 designation for this condition: EMA/OD/091/13 Recombinant human insulin receptor monoclonal antibody-fused iduronate 2-sulfatase

2.2.6. - EMA/OD/090/15

Treatment of acute lymphoblastic leukaemia

Action: For adoption

Documents tabled:

Draft Summary report

COMP coordinator's comments

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

EMEA/OD/063/04 L-Asparaginase, EMEA/OD/015/05 Nelarabine, EMEA/OD/074/05 Dasatinib, EMEA/OD/033/06 L-asparaginase encapsulated in erythrocytes, EMEA/OD/070/06 Forodesine hydrochloride, EMEA/OD/065/07 Mercaptopurine (oral liquid), EMEA/OD/064/07 Methotrexate (oral liquid), EMEA/OD/002/08 Vincristine sulphate liposomes, EMEA/OD/114/08 Mercaptopurine (oral suspension), EMA/OD/097/10 Recombinant human histone H1.3 and recombinant human N-bis-met-histone H1.3, EMEA/OD/029/09 Blinatumomab, EMEA/OD/084/09 6-thioguanine (oral liquid), EMEA/OD/122/09 Benzamide, 3-(2-imidazo[1,2-b]pyridazin-3-ylethynyl)-4-methyl-N-[4-[(4-methyl-1-piperazinyl)methyl]-3-(trifluoromethyl)phenyl], EMA/OD/168/10 Pegylated recombinant Erwinia chrysanthemi L-asparaginase, EMA/OD/001/11 Allogeneic T cells encoding an exogenous thymidine kinase gene, EMA/OD/143/13 (2R,3R,4S,5R)-2-(6-amino-9H-purin-9-yl)-5-((((1r,3S)-3-(2-(5-(tert-butyl)-1Hbenzo[d]imidazol-2-yl)ethyl)cyclobutyl)(isopropyl) amino)methyl)tetrahydrofuran-3,4-diol, EMA/OD/120/14 Allogeneic CD34+ cells expanded ex-vivo with an aryl hydrocarbon receptor antagonist
Designations withdrawn: EMEA/OD/022/03 Aplidine, EMEA/OD/038/05 Imatinib mesilate, EMEA/OD/067/08 Allogeneic ex vivo expanded umbilical cord blood cells

2.2.7. - EMA/OD/077/15

Treatment of mantle cell lymphoma

Action: For adoption

Documents tabled:

Draft Summary report

COMP coordinator's comments

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

2.2.8. - EMA/OD/078/15

Treatment of primary mediastinal large B-cell lymphoma

Action: For adoption

Documents tabled:

Draft Summary report

COMP coordinator's comments

2.2.9. - EMA/OD/066/15

Treatment of Dravet Syndrome

Action: For adoption

Documents tabled:

Draft Summary report

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

2.2.10. - EMA/OD/065/15

Treatment of Lennox-Gastaut Syndrome

Action: For adoption

Documents tabled:
Draft Summary report

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

2.2.11. - EMA/OD/060/15

Treatment of acute radiation syndrome

Action: For adoption

Documents tabled:
Draft Summary report

2.2.12. - EMA/OD/068/15

Treatment of cystic fibrosis

Action: For adoption

Documents tabled:
Draft Summary report
COMP coordinator's comments

Notes:
There have been 36 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, EMA/OD/024/10 3-(6-(1-(2,2-difluorobenzo [d] [1,3] dioxol-5-yl)cyclopropanecarboxamido)-3-methylpyridin-2-yl)benzoic acid, EMEA/OD/039/04 Dexamethasone sodium phosphate encapsulated in human erythrocytes, EMEA/OD/053/04 Alpha-1 antitrypsin (inhalation use), 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/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/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/032/14 Lumacaftor/ivacaftor, 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
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2.2.13. - EMA/OD/079/15

Treatment of retinal artery occlusion

Action: For adoption

Documents tabled:
Draft Summary report

2.2.14. - EMA/OD/082/15

Treatment of marginal zone lymphoma

Action: For adoption

Documents tabled:
Draft Summary report
COMP coordinator's comments

Notes:
There have been 2 designations for this condition: EMA/OD/284/14 Lenalidomide, EMA/OD/014/15 obinutuzumab

2.2.15. - EMA/OD/083/15

Treatment of cervical cancer

Action: For adoption

Documents tabled:
Draft Summary report

2.2.16. - EMA/OD/088/15

Treatment of aneurysmal subarachnoid hemorrhage (aSAH)

Action: For adoption

Documents tabled:
Draft Summary report

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

2.2.17. - EMA/OD/062/15

Treatment of snakebite envenomation

Action: For adoption

Documents tabled:
Draft Summary report

2.2.18. - EMA/OD/061/15

Treatment of cystic fibrosis

Action: For adoption

Documents tabled:
Draft Summary report
COMP coordinator's comments

Notes:
There have been 36 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, EMA/OD/024/10 3-(6-(1-(2,2-difluorobenzo [d] [1,3] dioxol-5-yl)cyclopropanecarboxamido)-3-methylpyridin-2-yl)benzoic acid, EMEA/OD/039/04 Dexamethasone sodium phosphate encapsulated in human erythrocytes, EMEA/OD/053/04 Alpha-1 antitrypsin (inhalation use), 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/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/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/032/14 Lumacaftor/ivacaftor, 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
Designations withdrawn: EMEA/OD/064/00 8-cyclopentyl-1, 3-dipropylxanthine, 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/018/03 Engineered protein inhibitor of human neutrophil elastase, EMEA/OD/075/02 Amiloride hydrochloride dihydrate, EMEA/OD/118/05 Glutathione, EMEA/OD/054/05 Heparin sodium (inhalation use), EMEA/OD/024/08 Levofloxacin hemihydrate

2.2.19. - EMA/OD/067/15

Treatment of progressive supranuclear palsy

Action: For adoption

Documents tabled:

Draft Summary report

COMP coordinator's comments

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

2.2.20. - EMA/OD/075/15

Treatment of hereditary angioedema

Action: For adoption

Documents tabled:

Draft Summary report

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

2.2.21. - EMA/OD/073/15

Treatment of pulmonary hypertension associated with idiopathic interstitial pneumonia

Action: For adoption

Documents tabled:

Draft Summary report

2.2.22. - EMA/OD/092/15

Treatment of chronic iron overload requiring chelation therapy

Action: For adoption

Documents tabled:

Draft Summary report

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

2.2.23. - EMA/OD/071/15

Treatment of primary graft dysfunction following lung transplantation

Action: For adoption

Documents tabled:
Draft Summary report
COMP coordinator's comments

2.2.24. - EMA/OD/089/15

Treatment of acute myeloid leukaemia

Action: For adoption

Documents tabled:
Draft Summary report

Notes:
There have been 40 designations for this condition: EMEA/OD/022/00 Gemtuzumab ozogamicin, EMA/OD/044/10 Allogeneic T cells encoding an exogenous TK gene, EMEA/OD/028/04 Midostaurin, EMEA/OD/051/04 Homoharringtonine, EMEA/OD/098/04 Tipifarnib, EMEA/OD/094/04 Histamine dihydrochloride, EMEA/OD/066/05 1,2-bis(methylsulphonyl)-1-(2-chloroethyl)-2-[(methylamino)carbonyl]hydrazine, EMEA/OD/100/05 zosuquidar trihydrochloride, EMEA/OD/004/06 Decitabine, EMEA/OD/056/06 Antisense oligonucleotide 5'-d[P-Thio] (CCCTG CTCCC CCCTG GCTCC)-3' (see comments box for cenersen sodium), EMEA/OD/049/07 5'-O-(trans-9"-octadecenoyl)-1-ß-D-arabinofuranosyl cytosine, EMEA/OD/087/07 Recombinant human histone H1.3 and recombinant human N-bis-met-histone H1.3, EMEA/OD/085/07 Azacitidine, EMEA/OD/099/07 N- (2-Amino-phenyl)-4-[(4-pyridin-3-yl-pyrimidin-2-ylamino)-methyl] benzamide, EMEA/OD/118/07 Ribonucleotide reductase R2 specific phosphorothioate oligonucleotide, EMEA/OD/015/08 Sapacitabine, EMEA/OD/048/08 Daunorubicin (liposomal), EMEA/OD/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/105/08 N-(5-tert-Butylisoxazol-3-yl)-N'-{4-[7-(2-(morpholin-4-yl)ethoxy) imidazo[2,1-b][1,3]benzothiazol-2-yl]phenyl}urea di-hydrochloride salt, EMEA/OD/028/09 Tosedostat, EMEA/OD/091/09 1-Cyclopropyl-3-[3-(5-morpholin-4-ylmethyl-1H-benzoimidazol-2-yl)-1H-pyrazol-4-yl]-urea, EMEA/OD/147/09 2-methoxymethyl-2-hydroxymethyl-1-azabicyclo[2,2,2]octan-3-one, EMA/OD/094/10 N-[(2S)-2,3-dihydroxypropyl]-3-[(2-fluoro-4-iodophenyl) amino] isonicotinamide hydrochloride, EMA/OD/161/10 Allogeneic bone marrow stem cells treated ex vivo with 16,16-dimethyl prostaglandin E2, EMA/OD/156/10 Allogeneic umbilical cord blood cells treated ex vivo with 16,16-dimethyl prostaglandin E2, EMA/OD/101/11 Allogeneic human dendritic cells derived from a CD34+ progenitor cell line, EMA/OD/070/11 Liposomal combination of cytarabine and daunorubicin, EMA/OD/158/11 Vosaroxin, EMA/OD/105/12 Liposomal daunorubicin, EMA/OD/167/12 L-asparaginase encapsulated in erythrocytes, EMA/OD/064/13 trans-N1-((1R,2S)-2-phenylcyclopropyl)cyclohexane-1,4-diamine bis-hydrochloride, EMA/OD/141/13 (2R,3R,4S,5R)-2-(6-amino-9H-purin-9-yl)-5-((((1r,3S)-3-(2-(5-(tert-butyl)-1Hbenzo[d]imidazol-2-yl)ethyl)cyclobutyl)(isopropyl) amino)methyl)tetrahydrofuran-3,4-diol, EMA/OD/181/13 Volasertib, EMA/OD/100/14 4-{[(2R,3S,4R,5S)-4-(4-Chloro-2-fluoro-phenyl)-3-(3-chloro-2-fluoro-phenyl)-4-cyano-5-(2,2-dimethyl-propyl)-pyrrolidine-2-carbonyl]-amino}-3-methoxy-benzoic acid, EMA/OD/258/14 Ulocuplumab, EMA/OD/061/14 (Z)-3-(3-(3,5-bis(trifluoromethyl)phenyl)-1H-1,2,4-triazol-1-yl)-N'-(pyrazin-2-yl)acrylohydrazide, EMA/OD/103/14 Donor T lymphocytes depleted ex vivo of host alloreactive T cells using photodynamic treatment, EMA/OD/175/14 Allogeneic ex vivo-generated natural killer cells from CD34+ umbilical cord blood progenitor cells, EMA/OD/240/14 Alvocidib, EMA/OD/188/14 Allogeneic, umbilical cord blood-derived, ex vivo-expanded, haematopoietic CD133+ cells / allogeneic, umbilical cord blood-derived, non-expanded, haematopoietic CD133- cells

Designations withdrawn: EMEA/OD/065/02 2-chloro-9-[2-deoxy-2-fluoro-ß-D-arabinofuranosyl]adenine, EMEA/OD/059/04 Val-Leu-Gln-Glu-Leu-Asn-Val-Thr-Val (Pr1 nanopeptide, sequence 169-177, of proteinase 3), EMEA/OD/045/05 Troxacitabine, EMEA/OD/018/06 Human monoclonal antibody against inhibitory killer cell lg-like receptors (1-7 F9), EMEA/OD/020/06 Lestaurtinib, EMEA/OD/024/07 Arsenic trioxide, EMEA/OD/069/07 Amonafide L-malate, EMEA/OD/118/08 Lintuzumab, EMEA/OD/090/08 Allogeneic ex vivo expanded umbilical cord blood cells, EMEA/OD/016/09 26 base single stranded phosphodiester DNA oligonucleotide, EMEA/OD/132/09 (1S, 2S, 3R, 4R)-3-(5-Fluoro-2-(3-methyl-4-(4-methylpiperazin-1-yl)-phenylamino)-pyrimidin-4-ylamino)-bicyclo[2.2.1]hept-5-ene-2-carboxamide benzoate), EMA/OD/023/10 1-[2-(Benzo[1,2,5]thiadiazol-5-ylamino)-6-(2,6-dichloro-phenyl)-pyrido[2,3-d]pyrimidin-7-yl]-3-tert-butyl-urea, EMA/OD/067/11 1-(4-{4-amino-7-[1-(2-hydroxyethyl)-1H- pyrazol-4-yl]thieno[3,2-c]pyridin-3-yl}phenyl)-3-(3-fluorophenyl)urea

2.2.25. - EMA/OD/063/15

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)

2.3. Revision of the COMP opinions

None.

2.4. COMP opinions adopted via written procedure following previous meeting

None.

2.5. Appeal

None.

2.6. Nominations

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

Action: For adoption

Document tabled:

OMPD applications - appointment of coord. at the 14-16 July 2015 COMP meeting

2.7. Evaluation on-going

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

Action: For information

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

3. Requests for protocol assistance with significant benefit question

3.1. Ongoing procedures

3.1.1. -

Treatment of follicular lymphoma

Action: For adoption

3.1.2. -

Treatment of graft-versus-host disease

Action: For adoption

3.1.3. -

Treatment of sickle cell disease

Action: For adoption

3.2. Finalised letters

3.2.1. -

Treatment of Niemann-Pick disease

Action: For information

3.2.2. -

Treatment of Graft-versus-Host disease

Action: For information

3.2.3. -

Treatment of Urea Cycle Disorders:

a) treatment of ornithine transcarbamylase deficiency;

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

c) treatment of citrullinaemia type 1;

d) treatment of argininosuccinic aciduria;

e) treatment of hyperargininaemia;

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

g) treatment of citrullinaemia type 2;

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

Action: For information

3.2.4. -

Treatment of pancreatic cancer condition

Action: For information

3.2.5. -

Treatment of Dravet syndrome

Action: For information

3.3. New requests

3.3.1. -

Treatment of systemic sclerosis

Action: For information

3.3.2. -

Treatment of ovarian cancer

Action: For information

3.3.3. -

Treatment of acromegaly

Action: For information

3.3.4. -

Treatment of Prader-Willi syndrome

Action: For information

4. Review of orphan designation for orphan medicinal products for marketing authorisation

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

4.1.1. FARYDAK - panobinostat - EMEA/H/C/003725, EMA/OD/113/12, EU/3/12/1063

Novartis Europharm Ltd; Treatment of multiple myeloma

Action: For adoption

Documents tabled:
Draft report on review of OMPD
CHMP AR

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

4.1.2. Kanuma - sebelipase alfa - EMEA/H/C/004004, EMA/OD/104/10, EU/3/10/827

Synageva BioPharma Ltd; Treatment of lysosomal acid lipase deficiency

Action: For adoption

Documents tabled:
Draft report on review of OMPD
CHMP AR

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

4.1.3. Raxone - idebenone - EMEA/H/C/003834, EMA/OD/076/06, EU/3/07/434

Santhera Pharmaceuticals (Deutschland) GmbH; Treatment of Leber's hereditary optic neuropathy

Action: For adoption

Documents tabled:
Draft report on review of OMPD
CHMP AR

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

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

Alexion Europe SAS; Treatment of hypophosphatasia

Action: For adoption

Documents tabled:
Draft report on review of OMPD
CHMP AR

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

4.1.5. Heparesc - human heterologous liver cells - ATMP - EMEA/H/C/003750

Cytonet GmbH&Co KG;

a) treatment of carbamoyl-phosphate synthase-1 deficiency (EMA/OD/108/10, EU/3/10/821)

b) treatment of ornithine-transcarbamylase deficiency (EMEA/OD/042/07, EU/3/07/470)

c) treatment of citrullinaemia type 1 (EMA/OD/105/10, EU/3/10/818)

d) treatment of hyperargininaemia (EMA/OD/106/10, EU/3/10/819)

e) treatment of argininosuccinic aciduria (EMA/OD/107/10, EU/3/10/820)

Action: For information

Notes:
Status of the procedure at the CHMP: CHMP adopted negative opinion in June 2015.

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

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

Basilea Medical Ltd;

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

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

Action: For discussion

Documents tabled:
Draft report on review of OMPD

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

Baxter AG; Treatment of haemophilia A

Action: For discussion

Document tabled:
Draft report on review of OMPD

4.2.3. - recombinant l-asparaginase – EMEA/H/C/002661

medac Gesellschaft fuer klinische Spezialpraeparate mbH; Treatment of acute lymphoblastic leukaemia

Action: For discussion

Document tabled:
Draft report on review of OMPD

4.3. On-going procedures

4.3.1. List of on-going procedures

Action: For information

5. Organisational, regulatory and methodological matters

5.1. Mandate and organisation of the COMP

5.1.1. Strategic Review & Learning meetings

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

Action: For information

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

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

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

5.2. Coordination with EMA Scientific Committees or CMDh-v

5.2.1. Enhanced early dialogue to foster development and facilitate accelerated assessment

Action: comments should be sent

Document tabled:
concept and features

5.2.2. Guidance on Uncertainty in EFSA Scientific Assessment

Action: comments should be sent

Document tabled:
Guidance on Uncertainty in EFSA Scientific Assessment

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

5.3.1. Significant Benefit Working Group

Proposed meeting time 15 July at 17:00-18:30, room: 3E

Action: For information

Documents tabled:

Draft SBWG agenda for 15 July 2015
SBWG minutes for 18 June 2015

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

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

EMA Human Scientific Committees' Working Parties with Patients' and Consumers' Organisations (PCWP) and Healthcare Professionals' Organisations (HCPWP) joint meeting – 17 September 2015

Action: For information

Documents tabled:
Draft PCWP and HCPWP joint workshop on risk minimisation measures agenda
Draft PCWP and HCPWP joint meeting agenda

5.4. Cooperation within the EU regulatory network

5.4.1. European Commission

None.

5.5. Cooperation with International Regulators

5.5.1. Food and Drug Administration (FDA)

EMA/FDA teleconference on Orphan Medicines – 9 June 2015

Action: for information

Document tabled:
Agenda

5.5.2. Ministry of Health, Labour and Welfare (MHLW) - Pharmaceuticals and Medical Devices Agency (PMDA)

EMA/MHLW-PMDA teleconference on Orphan Medicines – 23 June 2015

Action: for information

Document tabled:
Agenda

5.5.3. EMA/MHLW/PMDA/NIBIOHN Orphan Drugs/ Orphan Devices Workshop

Action: for information

Notes: Co-hosted by NINIOHN and PMDA 7-9 September 2015

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

None.

5.7. COMP work plan

Work plan 2015 tracking and drafting 2016

Action: for information

5.8. Planning and reporting

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

Action: For information

5.8.2. Overview of orphan marketing authorisations/applications

Action: For information

5.8.3. Meeting dates

Action: For information

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

6. Any other business

6.1. Eurordis Summer School

Action: For information