11 February 2016
EMA/COMP/130661/2016
Procedure Management and Committees Support Division

Committee for Orphan Medicinal Products (COMP)

Draft agenda for the meeting on 16-18 February 2016

Chair: Bruno Sepodes – Vice-Chair: Lesley Greene

16 February 2016, 09:00-19:00, room 2F

17 February 2016, 08:30-19:00, room 2F

18 February 2016, 08:30-17:00, room 2F

Health and safety information

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

Disclaimers

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

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

Note on access to documents

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

30 Churchill Place ● Canary Wharf ● London E14 5EU ● United Kingdom
Telephone +44 (0)20 3660 6000 **Facsimile** +44 (0)20 3660 5555
Send a question via our website www.ema.europa.eu/contact

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 16-18 February 2016. See February 2016 COMP minutes (to be published post March 2016 COMP meeting).

1.2. Adoption of agenda

COMP agenda for 16-18 February 2016.

1.3. Adoption of the minutes

COMP minutes for 19-21 January 2016.

2. Applications for orphan medicinal product designation

2.1. For opinion

2.1.1. EMA/OD/159/15

Treatment of soft tissue sarcoma

Action: For adoption, Oral explanation to be held on 16 February 2016 at time 09:30

Documents tabled:
Draft Summary report with response to LoQs

Notes:
There are currently 8 designations for this condition: EMEA/OD/001/01 Ecteinascidin 743, EMA/OD/155/11 Yttrium (90Y)-DTPA-radiolabelled chimeric monoclonal antibody against frizzled homologue 10, EMEA/OD/042/06 Doxorubicin hydrochloride (liposomal), EMA/OD/110/11 (1-methyl-2-nitro-1H-imidazole-5-yl)methyl N,N'-bis(2-bromoethyl) diamidophosphate, EMA/OD/041/13 Genetically modified serotype 5/3 adenovirus coding for granulocyte macrophage colony-stimulating factor, EMA/OD/190/13 Doxorubicin(6-maleimidocaproyl)hydrazone, EMA/OD/266/14 Olaratumab, EMA/OD/184/15 (S)-N-(5-((R)-2-(2,5-difluorophenyl)pyrrolidin-1-yl)pyrazolo[1,5-a]pyrimidin-3-yl)-3-hydroxypyrrolidine-1-carboxamide hydrogen sulfate

Designations withdrawn: EMEA/OD/059/03 N-acetylsarcosyl-glycyl-L-valyl-D-alloisoleucyl-L-threonyl-L-norvalyl-L-isoleucyl-L-arginyl-L-prolyl-N-ethylamide, EMEA/OD/050/05 (1R, 2R, 4S)-4-{(2R)-2-[(3S,6R,7E,9R,10R,12R,14S,15E,17E,19E,21S,23S,26R, 27R,34aS)-9,27-dihydroxy-10,21-dimethoxy-6,8,12,14,20,26-hexamethyl-1,5,11,28,29-pentaoxo-1,4,5,6,9,10,11,12,13,14,21,22,23,24,25,26,27,28,29,31,32,33,34,34a-tetra-co, EMEA/OD/071/05 Brostallicin, EMEA/OD/083/06 Fenretinide, EMEA/OD/044/08 Palifosfamide, EMA/OD/141/10 Ombrabulin, EMA/OD/160/15 Sindbis virus envelope pseudotyped lentiviral vector encoding New York esophageal squamous cell carcinoma-1, EMA/OD/121/11 Doxorubicin(6-maleimidocaproyl)hydrazine, EMEA/OD/071/05 Brostallicin,

EMEA/OD/059/03 N-acetylsarcosyl-glycyl-L-valyl-D-alloisoleucyl-L-threonyl-L-norvalyl-L-isoleucyl-L-arginyl-L-prolyl-N-ethylamide, EMEA/OD/036/09 (-)-trans-3-(5,6-dihydro-4H-pyrrolo [3,2,1-ij] quinolin-1yl)-4(1H-indol-3-yl) pyrrolidine-2, 5-dione

2.1.2. EMA/OD/238/15

Treatment of soft tissue sarcoma

Action: For adoption, Oral explanation to be held on 16 February 2016 at time 09:30

Documents tabled:
Draft Summary report with response to LoQs

Notes:
There are currently 8 designations for this condition: Please 2.1.1.

2.1.3. EMA/OD/211/15

Treatment of gastro-entero-pancreatic neuroendocrine tumours

Action: For adoption, Oral explanation to be held on 16 February 2016 at time 18:00

Documents tabled:
Draft Summary report with response to LoQs

Notes:
There are currently 3 designations for this condition: EMEA/OD/093/07 Lutetium (177Lu)-N-[(4,7,10-Tricarboxymethyl-1,4,7,10-tetraazacyclododec-1-yl)acetyl]-D-phenylalanyl-L-cysteinyl-L-tyrosyl-D-tryptophanyl-L-lysyl-L-threoninyl-L-cysteinyl-L-threonine-cyclic(2-7)disulfide, EMA/OD/196/13 Lutetium (177Lu) edotreotide, EMA/OD/147/15 (R)-1-[1-(4-acetoxy-3,3-dimethyl-2-oxo-butyl)-2-oxo-5-(pyridin-2-yl)-2,3-dihydro-1H-benzo[e][1,4]diazepin-3-yl]-3-(3-methylamino-phenyl)-urea

Designation withdrawn: EMEA/OD/040/07 Everolimus

2.1.4. EMA/OD/179/15

Treatment of pulmonary arterial hypertension

Action: For adoption, Oral explanation to be held on 17 February 2016 at time 09:30

Documents tabled:
Draft Summary report with response to LoQs

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

2.1.5. EMA/OD/198/15

Treatment of acute myeloid leukaemia

Action: For information

Documents tabled:
Withdrawal request of 27 January 2016

Notes:
Withdrawn
There are currently 45 designations for this condition: EMEA/OD/022/00 Gemtuzumab ozogamicin, EMA/OD/044/10 Allogeneic T cells encoding an exogenous TK gene, EMEA/OD/028/04 Midostaurin, EMEA/OD/051/04 Homoharringtonine, EMEA/OD/098/04 Tipifarnib, EMEA/OD/094/04 Histamine dihydrochloride, EMEA/OD/066/05 1,2-bis(methylsulphonyl)-1-(2-chloroethyl)-2-[(methylamino)carbonyl]hydrazine, EMEA/OD/100/05 zosuquidar trihydrochloride, EMEA/OD/004/06 Decitabine, EMEA/OD/056/06 Antisense oligonucleotide 5'-d[P-Thio] (CCCTG CTCCC CCCTG GCTCC)-3' (see comments box for cenersen sodium), EMEA/OD/049/07 5'-O-(trans-9"-octadecenoyl)-1-ß-D-arabinofuranosyl cytosine, EMEA/OD/087/07 Recombinant human histone H1.3 and recombinant human N-bis-met-histone H1.3, EMEA/OD/085/07 Azacitidine, EMEA/OD/099/07 N- (2-Amino-phenyl)-4-[(4-pyridin-3-yl-pyrimidin-2-ylamino)-methyl] benzamide, EMEA/OD/118/07 Ribonucleotide reductase R2 specific phosphorothioate oligonucleotide, EMEA/OD/015/08 Sapacitabine, EMEA/OD/048/08 Daunorubicin (liposomal), EMEA/OD/105/08 N-(5-tert-Butylisoxazol-3-yl)-N'-{4-[7-(2-(morpholin-4-yl)ethoxy) imidazo[2,1-b][1,3]benzothiazol-2-yl]phenyl}urea di-hydrochloride salt, EMEA/OD/028/09 Tosedostat, EMEA/OD/091/09 1-Cyclopropyl-3-[3-(5-morpholin-4-ylmethyl-1H-benzoimidazol-2-yl)-1H-pyrazol-4-yl]-urea, EMEA/OD/147/09 2-methoxymethyl-2-hydroxymethyl-1-azabicyclo[2,2,2]octan-3-one, EMA/OD/094/10 N-[(2S)-2,3-dihydroxypropyl]-3-[(2-fluoro-4-iodophenyl) amino] isonicotinamide hydrochloride, EMA/OD/161/10 Allogeneic bone marrow stem cells treated ex vivo with 16,16-dimethyl prostaglandin E2, EMA/OD/156/10 Allogeneic umbilical cord blood cells treated ex vivo with 16,16-dimethyl prostaglandin E2, EMA/OD/101/11 Allogeneic human dendritic cells derived from a CD34+ progenitor cell line, EMA/OD/070/11 Liposomal combination of cytarabine and daunorubicin, EMA/OD/158/11 Vosaroxin, EMA/OD/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, EMA/OD/045/15 inecalcitol, EMA/OD/037/15 2-((3-((4-((3-aminopropyl)amino)butyl)amino)propyl)amino)-N-((5S,5aS,8aR,9R)-9-(4-hydroxy-3,5-dimethoxyphenyl)-8-oxo-5,5a,6,8,8a,9-hexahydrofuro[3',4':6,7]naphtho[2,3-

d][1,3]dioxol-5-yl)acetamide, tetrahydrochloride, EMA/OD/089/15 CD33-directed antibody-drug conjugate consisting of an antibody conjugated to a DNA cross-linking pyrrolobenzodiazepine dimer drug, EMA/OD/112/15 Recombinant human interleukin-3 truncated diphtheria toxin fusion protein, EMA/OD/145/15 Humanised monoclonal antibody of the IgG4 kappa isotype targeting CD47, EMA/OD/165/15 Sodium (2R,3S,5R)-5-(4-amino-2-oxo-1,3,5-triazin-1(2H)-yl)-2-(hydroxymethyl)tetrahydrofuran-3-yl ((2R,3S,5R)-5-(2-amino-6-oxo-1H-purin-9(6H)-yl)-3-hydroxytetrahydrofuran-2-yl)methyl phosphate, EMA/OD/144/15 Combretastatin A1-diphosphate

Designations withdrawn: EMEA/OD/065/02 2-chloro-9-[2-deoxy-2-fluoro-ß-D-arabinofuranosyl]adenine, EMEA/OD/059/04 Val-Leu-Gln-Glu-Leu-Asn-Val-Thr-Val (Pr1 nanopeptide, sequence 169-177, of proteinase 3), EMEA/OD/045/05 Troxacitabine, EMEA/OD/018/06 Human monoclonal antibody against inhibitory killer cell lg-like receptors (1-7 F9), EMEA/OD/020/06 Lestaurtinib, EMEA/OD/024/07 Arsenic trioxide, EMEA/OD/069/07 Amonafide L-malate, EMEA/OD/060/08 2-[[3-({4-[(5-{2-[(3-Fluorophenyl)amino]-2-oxoethyl}-1H-pyrazol-3-yl)amino]-quinazolin-7-yl}oxy)propyl](ethyl)amino]ethyl dihydrogen phosphate trihydrate, EMEA/OD/118/08 Lintuzumab, EMEA/OD/090/08 Allogeneic ex vivo expanded umbilical cord blood cells, EMEA/OD/016/09 26 base single stranded phosphodiester DNA oligonucleotide, EMEA/OD/132/09 (1S, 2S, 3R, 4R)-3-(5-Fluoro-2-(3-methyl-4-(4-methylpiperazin-1-yl)-phenylamino)-pyrimidin-4-ylamino)-bicyclo[2.2.1]hept-5-ene-2-carboxamide benzoate), EMA/OD/023/10 1-[2-(Benzo[1,2,5]thiadiazol-5-ylamino)-6-(2,6-dichloro-phenyl)-pyrido[2,3-d]pyrimidin-7-yl]-3-tert-butyl-urea, EMA/OD/067/11 1-(4-{4-amino-7-[1-(2-hydroxyethyl)-1H- pyrazol-4-yl]thieno[3,2-c]pyridin-3-yl}phenyl)-3-(3-fluorophenyl)urea, EMA/OD/105/12 Liposomal daunorubicin

2.1.6. EMA/OD/196/15

Treatment of chronic lymphocytic leukaemia / small lymphocytic lymphoma

Action: For adoption, Oral explanation to be held on 17 February 2016 at time 12:00

Documents tabled:
Draft Summary report with response to LoQs

Notes:
There is currently 1 designation for this condition: EMA/OD/109/13 inecalcitol

Designation withdrawn: EMA/OD/151/13 N-(3-(5-fluoro-2-(4-(2-methoxyethoxy)phenylamino)pyrimidin-4-ylamino) phenyl)acrylamide benzenesulfonic acid salt

2.1.7. EMA/OD/214/15

Treatment of gastro-entero-pancreatic neuroendocrine tumours

Action: For adoption, Oral explanation to be held on 17 February 2016 at time 14:30

Documents tabled:
Draft Summary report with response to LoQs

Notes:
There are currently 3 designations for this condition: EMEA/OD/093/07 Lutetium (177Lu)-N-[(4,7,10-Tricarboxymethyl-1,4,7,10-tetraazacyclododec-1-yl)acetyl]-D-phenylalanyl-L-cysteinyl-L-tyrosyl-D-tryptophanyl-L-lysyl-L-threoninyl-L-cysteinyl-L-threonine-cyclic(2-7)disulfide, EMA/OD/196/13 Lutetium (177Lu) edotreotide, EMA/OD/147/15 (R)-1-[1-(4-acetoxy-3,3-dimethyl-2-oxo-butyl)-2-oxo-5-(pyridin-2-yl)-2,3-dihydro-1H-benzo[e][1,4]diazepin-3-yl]-3-(3-methylamino-phenyl)-urea

Designation withdrawn: EMEA/OD/040/07 Everolimus

2.1.8. EMA/OD/209/15

Treatment of graft rejection following solid organ transplantation

Action: For adoption, Oral explanation to be held on 17 February 2016 at time 15:30

Documents tabled:
Draft Summary report with response to LoQs

2.1.9. EMA/OD/176/15

Treatment of acute lymphoblastic leukemia

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

Documents tabled:
Draft Summary report with response to LoQs

Notes:
There have been 20 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, 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/038/05 Imatinib mesilate, EMEA/OD/067/08 Allogeneic ex vivo expanded umbilical cord blood cells

2.1.10. EMA/OD/200/15

Diagnosis of hepatocellular carcinoma

Action: For adoption, Oral explanation to be held on 17 February 2016 at time 18:00

Documents tabled:
Draft Summary report with response to LoQs

2.1.11. EMA/OD/201/15

Diagnosis of glioma

Action: For adoption, Oral explanation to be held on 17 February 2016 at time 18:00

Documents tabled:
Draft Summary report with response to LoQs

Notes:
There are currently 2 designations for this condition: EMEA/OD/015/06 4-[123I] iodo-L-phenylalanine, EMA/OD/280/14 Fluciclovine (18F)

2.1.12. EMA/OD/181/15

Treatment of monogenic diabetes

Action: For adoption, Oral explanation to be held on 18 February 2016 at time 09:30

Documents tabled:
Draft Summary report with response to LoQs

2.1.13. EMA/OD/210/15

Treatment of Epstein-Barr Virus-associated lymphoproliferative disorder following allogeneic haematopoietic cell transplant

Action: For adoption

Documents tabled:
Draft Summary report with response to LoQs

Notes:
There is currently 1 designation for this condition: EMA/OD/247/14 Allogeneic CD4+ and CD8+ T lymphocytes ex vivo incubated with synthetic peptides of the viral antigens of cytomegalovirus, adenovirus and Epstein-Barr virus

Designation withdrawn: EMA/OD/095/14 Naturally occurring donor lymphocytes enriched for antigen-specific CD4+ and CD8+ T cells

2.1.14. EMA/OD/203/15

Treatment of cutaneous T-cell lymphoma

Action: For adoption

Documents tabled:
Draft Summary report with response to LoQs

Notes:
There are currently 11 designations for this condition: EMEA/OD/038/01 Denileukin diftitox, EMEA/OD/001/04 Human monoclonal antibody against CD4, EMEA/OD/001/05 (E)-(1S,4S,10S,21R)-7-[(Z)-ethylidene]-4,21-diisopropyl-2-oxa-12,13-dithia-5,8,20,23-tetraazabicyclo[8.7.6]tricos-16-ene-3,6,9,19,22-pentone, EMEA/OD/030/08 Miltefosine, EMEA/OD/135/09 Pralatrexate, EMA/OD/112/11 Chlormethine, EMA/OD/100/11 Brentuximab vedotin, EMA/OD/050/12 Naloxone hydrochloride dihydrate, EMA/OD/066/12 Recombinant anti-CD3-bi-single-chain-Fv-diphtheria toxin fusion protein, EMA/OD/084/14 Humanised IgG1 monoclonal antibody against human KIR3DL2, EMA/OD/033/15 Synthetic hypericin

Designations withdrawn: EMEA/OD/007/03 Adenovirus-Interferon gamma-coding DNA sequence, EMEA/OD/003/04 Suberolylanilide Hydroxamic acid, EMEA/OD/015/07 Panobinostat lactate

2.1.15. EMA/OD/195/15

Treatment of primary hyperoxaluria type 1

Action: For adoption

Documents tabled:
Draft Summary report with response to LoQs

Notes:
There are currently 2 designations for this condition: EMA/OD/123/11 Adeno-associated viral vector of serotype 5 containing the human alanine-glyoxylate aminotransferase gene, EMA/OD/052/15 Synthetic double-stranded RNA oligonucleotide specific to hydroxyacid oxidase 1 gene

2.2. For discussion / preparation for an opinion

2.2.1. EMA/OD/237/15

Treatment of lymphoplasmacytic lymphoma

Action: For adoption

Documents tabled:
Draft Summary report

Notes:
There is currently 1 designation for this condition: EMA/OD/185/13 ibrutinib

Designation withdrawn: EMA/OD/055/13 Idelalisib

2.2.2. EMA/OD/231/15

Treatment of mantle cell lymphoma

Action: For adoption

Documents tabled:
Draft Summary report

Notes:
There are currently 8 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, EMA/OD/077/15 Autologous T cells transduced with retroviral vector encoding an anti-CD19 CD28/CD3-zeta chimeric antigen receptor

2.2.3. EMA/OD/230/15

Treatment of haemophilia A

Action: For adoption

Documents tabled:
Draft Summary report

Notes:
There are currently 8 designations for this condition: EMA/OD/128/10 Pegylated B-domain-deleted sequence-modified recombinant human factor VIII, EMA/OD/132/10 Recombinant fusion protein linking human coagulation factor VIIa with human albumin (rVIIa-FP), EMA/OD/144/11 Pegylated recombinant factor VIII, EMA/OD/095/12 Humanised monoclonal IgG4 antibody against tissue factor pathway inhibitor, EMA/OD/039/14 Synthetic double-stranded siRNA oligonucleotide directed against antithrombin mRNA that is covalently linked to a ligand containing three N-acetylgalactosamine residues, EMA/OD/144/13 Humanised monoclonal modified IgG4 antibody with bispecific structure targeting factors IX, IXa, X and Xa, EMA/OD/069/14 Recombinant factor VIIa modified with three terminal repeats derived from the β chain of human chorionic gonadotropin, EMA/OD/123/14 A combination of H-Lys-Lys-Gly-Pro-Arg-Cys(SH)-Leu-Thr-Arg-Tyr-Tyr-Ser-Ser-Phe-Val-Asn-Met-Glu-Gly-Lys-Lys-OH and H-Lys-Lys-Gly-Asp-Asn-Ile-Met-Val-Thr-Phe-Arg-Asn-Gln-Ala-Ser-Arg-Pro-Tyr-Gly-Lys-Lys-OH

Designations withdrawn: EMEA/OD/031/09 Sequence-modified recombinant human factor VIIa, EMA/OD/030/10 Recombinant fusion protein consisting of human coagulation factor VIII attached to the Fc domain of human IgG1, EMA/OD/043/10 Recombinant porcine factor VIII (B domain deleted), EMA/OD/069/12 vatreptacog alfa (activated)

2.2.4. EMA/OD/227/15

Treatment of ornithine transcarbamylase deficiency

Action: For adoption

Documents tabled:
Draft Summary report

Notes:
There are currently 2 designations for this condition: EMEA/OD/101/07 Heterologous human adult liver derived stem cells, EMA/OD/026/11 Heterologous human adult liver-derived stem cells

Designation withdrawn: EMA/OD/097/11 Sodium phenylbutyrate

2.2.5. EMA/OD/212/15

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

2.2.6. EMA/OD/234/15

Prevention of Cytomegalovirus disease in patients with impaired cell-mediated immunity deemed at risk

Action: For adoption

Documents tabled:
Draft Summary report

Notes:
There is currently 1 designation for this condition: EMA/OD/090/10 (S)-{8-fluoro-2-2[4-(3-methoxyphenyl)-1-piperazinyl]-3-[2-methoxy-5-(trifluoromethyl)-phenyl]-3,4-dihydro-4-quinazolinyl} acetic acid

2.2.7. EMA/OD/192/15

Treatment of oesophageal cancer

Action: For adoption

Documents tabled:
Draft Summary report

Notes:
Designation withdrawn: EMEA/OD/002/02 bryostatin-1

2.2.8. EMA/OD/204/15

Treatment of pancreatic cancer

Action: For adoption

Documents tabled:
Draft Summary report

Notes:
There are currently 34 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/111/07 Chimeric antibody to mesothelin, 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

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/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

2.2.9. EMA/OD/187/15

Treatment of idiopathic intracranial hypertension

Action: For adoption

Documents tabled:
Draft Summary report

2.2.10. EMA/OD/215/15

Treatment of soft tissue sarcoma

Action: For adoption

Documents tabled:
Draft Summary report

Notes:
There are currently 8 designations for this condition: EMEA/OD/001/01 Ecteinascidin 743, EMA/OD/155/11 Yttrium (90Y)-DTPA-radiolabelled chimeric monoclonal antibody against frizzled homologue 10, EMEA/OD/042/06 Doxorubicin hydrochloride (liposomal), EMA/OD/110/11 (1-methyl-2-nitro-1H-imidazole-5-yl)methyl N,N'-bis(2-bromoethyl) diamidophosphate, EMA/OD/041/13 Genetically modified serotype 5/3 adenovirus coding for granulocyte macrophage colony-stimulating factor, EMA/OD/190/13 Doxorubicin(6-maleimidocaproyl)hydrazone, EMA/OD/266/14 Olaratumab, EMA/OD/184/15 (S)-N-(5-((R)-2-(2,5-difluorophenyl)pyrrolidin-1-yl)pyrazolo[1,5-a]pyrimidin-3-yl)-3-hydroxypyrrolidine-1-carboxamide hydrogen sulfate

Designations withdrawn: EMEA/OD/059/03 N-acetylsarcosyl-glycyl-L-valyl-D-alloisoleucyl-L-threonyl-L-norvalyl-L-isoleucyl-L-arginyl-L-prolyl-N-ethylamide, EMEA/OD/050/05 (1R, 2R, 4S)-4-{(2R)-2-[(3S,6R,7E,9R,10R,12R,14S,15E,17E,19E,21S,23S,26R, 27R,34aS)-9,27-dihydroxy-10,21-dimethoxy-6,8,12,14,20,26-hexamethyl-1,5,11,28,29-pentaoxo-1,4,5,6,9,10,11,12,13,14,21,22,23,24,25,26,27,28,29,31,32,33,34,34a-tetra-co, EMEA/OD/071/05 Brostallicin, EMEA/OD/083/06 Fenretinide, EMEA/OD/044/08 Palifosfamide, EMA/OD/141/10 Ombrabulin

2.2.11. EMA/OD/217/15

Prevention of short bowel syndrome

Action: For adoption

Documents tabled:
Draft Summary report

2.2.12. EMA/OD/236/15

Treatment of Smith-Magenis syndrome

Action: For adoption

Documents tabled:
Draft Summary report

Notes:
Designations withdrawn: EMA/OD/260/14 Tasimelteon

2.2.13. EMA/OD/228/15

Treatment of GNE myopathy

Action: For adoption

Documents tabled:
Draft Summary report

2.2.14. EMA/OD/235/15

Treatment of osteogenesis imperfecta

Action: For adoption

Documents tabled:
Draft Summary report

Notes:
There is currently 1 designation for this condition: EMA/OD/053/15 Human allogeneic bone-marrow-derived osteoblastic cells

2.2.15. EMA/OD/233/15

Treatment of acute myeloid leukaemia

Action: For adoption

Documents tabled:
Draft Summary report

Notes:
There are currently 45 designations for this condition: Please see 2.1.5.

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 16-18 February 2016 COMP meeting

2.7. Evaluation on-going

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

Action: For information

Notes:
See 5.8.1. Table 6. Evaluation Ongoing.

3. Requests for protocol assistance with significant benefit question

3.1. Ongoing procedures

3.1.1. -

Diagnosis of gastro-entero-pancreatic neuroendocrine tumours

Action: For adoption

3.2. Finalised letters

3.2.1. -

Treatment of Niemann-Pick disease, type C

Action: For information

3.2.2. -

Treatment of cytomegalovirus disease in patients with impaired cell mediated immunity

Action: For information

3.2.3. -

Treatment of advanced ovarian cancer

Action: For information

3.3. New requests

3.3.1. -

Treatment of pyruvate kinase deficiency

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. Wakix - 1-{3-[3-(4-chlorophenyl)propoxy]propyl}piperidine, hydrochloride - EMEA/OD/087/06, EU/3/07/459, EMEA/H/C/002616

Bioprojet; Treatment of narcolepsy

Action: For adoption, Oral explanation to be held on 16 February 2016 at time 14:30

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

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

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 adoption, Oral explanation to be held on 16 February 2016 at time 12:00

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

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

4.1.3. Revlimid – Lenalidomide - Type II variation - EMA/OD/078/11, EU/3/11/924, EMEA/H/C/000717/II/0079

Celgene Europe Limited; Treatment of mantle cell lymphoma

Action: For adoption, Oral explanation to be held on 16 February 2016 at time 11:00

Document tabled:
Draft report on review of OMPD
CHMP assessment report pending

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

4.1.4. Uptravi - selexipag - EMEA/OD/043/05, EU/3/05/316, EMEA/H/C/003774

Actelion Registration Ltd.; Treatment of pulmonary arterial hypertension and chronic thromboembolic pulmonary hypertension

Action: For adoption, Oral explanation to be held on 16 February 2016 at time 15:30

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

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

4.1.5. COAGADEX - factor X - EMEA/OD/044/07, EU/3/07/471, EMEA/H/C/003855

BIO PRODUCTS LABORATORY; Treatment of hereditary factor X deficiency

Action: For adoption, Oral explanation to be held on 16 February 2016 at time 17:00

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

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

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

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

Amicus Therapeutics UK Ltd; Treatment of Fabry disease

Action: For discussion

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

4.2.2. - albutrepenonacog alfa – EMEA/OD/117/09, EU/3/09/723, EMEA/H/C/003955

CSL Behring GmbH; Treatment of haemophilia B

Action: For discussion

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

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

Takeda Pharma A/S; multiple myeloma

Action: For discussion

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

4.2.4. eftrenonacog alfa – EMEA/OD/012/07, EU/3/07/453, EMEA/H/C/004142

Biogen Idec Ltd; Treatment of haemophilia B

Action: For discussion

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

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

5.1.1. –

Action: For information

6. Organisational, regulatory and methodological matters

6.1. Mandate and organisation of the COMP

6.1.1. Strategic Review & Learning meetings

None

6.1.2. Significant Benefit Working Group

Proposed meeting time on 17 February 2016 at time 13:30, room 2F

6.1.3. Preclinical Models Working Group

Proposed meeting time on 18 February 2016 at time 14:00, room 2F

6.1.4. Revised Standard Operating Procedures (SOPs)

3049 SOP – Orphan medicinal product designation EMA/358120/2005

3190 SOP – Review of orphan designation at the time of granting/varying a marketing authorisation EMA/71584/2007

Action: For information

Notes: Templates for COMP coordinator's comments / reader's guidance for both procedures are available in MMD/COMP/General/Templates.

6.2. Coordination with EMA Scientific Committees or CMDh-v

None

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

6.3.1. SAWP/COMP joint membership

Call of interest for a SAWP/COMP member

Action: For information

6.3.2. Patients' and Consumers' Working Party (PCWP) and Healthcare Professionals' Working Party (HCPWP) joint meetings

PCWP/HCPWP joint meeting – 17 September 2015

PCWP/HCPWP joint meeting - Session on communication and information on medicines – 08 March 2016

PCWP/HCPWP joint meeting – 09 March 2016

Report on EMA's workshop on risk minimisation measures -Towards optimising risk minimisation measures – 16 Sept 2015

Action: For information

Document tabled:
Minutes of the PCWP/HCPWP joint meeting – 17 September 2015
Agenda of the PCWP/HCPWP joint meeting – Session on communication and information on medicines - 08 March 2016
Draft Agenda of the PCWP/HCPWP joint meeting – 09 March 2016
EMA's workshop on risk minimisation measures -Report

6.3.3. Patients' and Consumers' Working Party (PCWP)

PCWP meeting with all eligible organisations – 26 November 2015

Action: For information

Document tabled:
Minutes of the PCWP meeting - 26 November 2015

6.3.4. Paediatric Committee (PDCO)

Proposed meeting time on 18 February 2016 at time 13:00, room 8A

Action: For information

Document tabled:
PDCO COMP Working Group Update of Current Activities

6.4. Cooperation within the EU regulatory network

6.4.1. European Commission

None

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

6.8.3. Data Gathering Initiative

Action: For information

Notes: Postponed from January COMP meeting.

7. Any other business

None