13 January 2016
EMA/COMP/844188/2015
Procedure Management and Committees Support Division

Committee for Orphan Medicinal Products (COMP)

Draft agenda for the meeting on 19-21 January 2016

Chair: Bruno Sepodes – Vice-Chair: Lesley Greene

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

20 January 2016, 08:30-19:00, room 2F

21 January 2016, 08:30-13:00, room 2F

Health and safety information

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

Disclaimers

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

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

Note on access to documents

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

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1. Introduction

1.1. Welcome and declarations of interest of members and experts

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

1.2. Adoption of agenda

COMP agenda for 19-21 January 2016.

1.3. Adoption of the minutes

COMP minutes for 8-10 December 2015.

2. Applications for orphan medicinal product designation

2.1. For opinion

2.1.1. - EMA/OD/183/15

Treatment of gastric cancer

Action: For information

Document tabled:
Withdrawal request of 30 December 2015

Notes:
Withdrawn
There are currently 6 designations for this condition: EMEA/OD/056/02 G17(9) gastrin-Diphtheria Toxoid Conjugate, EMEA/OD/044/06 Catumaxomab, EMA/OD/083/10 Chimeric monoclonal antibody against claudin-18 splice variant 2, EMA/OD/101/10 Tesetaxel, EMA/OD/030/12 Ramucirumab, EMA/OD/012/14 Rilotumumab

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

2.1.2. - EMA/OD/121/15

Treatment of amyotrophic lateral sclerosis

Action: For information

Documents tabled:
Withdrawal request of 30 December 2015

Notes:
Withdrawn

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

2.1.3. - EMA/OD/104/15

Treatment of C3 glomerulopathy

Action: For adoption, Oral explanation to be held on 20 January 2016 at time 18:00

Document tabled:
Draft Summary report with response to LoQs

2.1.4. - EMA/OD/182/15

Treatment of retinal detachment

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

Document tabled:
Draft Summary report with response to LoQs

2.1.5. - EMA/OD/186/15

Prevention of necrotising enterocolitis

Action: For information

Document tabled:
Withdrawal request of 4 January 2016

Notes:
Withdrawn
There are currently 2 designations for this condition: EMA/OD/112/13 Lactobacillus acidophilus / Bifidobacterium bifidum, EMA/OD/237/14 Lactobacillus reuteri

2.1.6. - EMA/OD/132/15

Treatment of Burkitt lymphoma

Action: For adoption, Oral explanation to be held on 20 January 2016 at time 11:00

Document tabled:
Draft Summary report with response to LoQs

Notes:
Designations withdrawn: EMA/OD/075/12 Sertraline

2.1.7. - EMA/OD/185/15

Treatment of partial deep dermal and full thickness burns

Action: For adoption, Oral explanation to be held on 20 January 2016 at time 12:00

Document tabled:
Draft Summary report with response to LoQs

Notes:
There has been 1 designation for this condition: EMEA/OD/012/02 Purified bromelain

2.1.8. - EMA/OD/189/15

Treatment of pantothenate kinase associated neurodegeneration

Action: For adoption, Oral explanation to be held on 20 January 2016 at time 14:30

Document tabled:
Draft Summary report with response to LoQs

Notes:
Designations withdrawn: EMA/OD/154/11 Deferiprone

2.1.9. - EMA/OD/190/15

Treatment of globoid cell leukodystrophy (Krabbe disease)

Action: For adoption, Oral explanation to be held on 20 January 2016 at time 15:30

Document tabled:
Draft Summary report with response to LoQs

Notes:
There is currently 1 designation for this condition: EMA/OD/042/11 Recombinant human galactocerebrosidase

2.1.10. - EMA/OD/177/15

Treatment of autoimmune haemolytic anaemia

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

Document tabled:
Draft Summary report with response to LoQs

2.1.11. - EMA/OD/180/15

Treatment of acute promyelocytic leukaemia

Action: For adoption

Document tabled:
Draft Summary report with response to LoQs

Notes:
Designations withdrawn: EMEA/OD/031/00 Arsenic trioxide, EMEA/OD/002/09 Tamibarotene

2.1.12. - EMA/OD/188/15

Treatment of dystrophic epidermolysis bullosa

Action: For adoption

Document tabled:
Draft Summary report with response to LoQs

Notes:
There is currently 1 designation for this condition: EMEA/OD/099/08 Skin equivalent graft genetically corrected with a COL7A1-encoding SIN retroviral vector

2.2. For discussion / preparation for an opinion

2.2.1. - EMA/OD/197/15

Treatment of Ebola virus disease

Action: For adoption

Document tabled:
Draft Summary report

Notes:
There are currently 2 designations for this condition: EMA/OD/250/14 Fibrinogen-coated albumin spheres, EMA/OD/310/14 Rintatolimod

2.2.2. - EMA/OD/196/15

Treatment of chronic lymphocytic leukaemia / small lymphocytic lymphoma

Action: For adoption

Document tabled:
Draft Summary report

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

2.2.3. - EMA/OD/210/15

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

Action: For adoption

Document tabled:
Draft Summary report

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

2.2.4. - EMA/OD/213/15

Treatment of retinitis pigmentosa

Action: For adoption

Document tabled:
Draft Summary report

Notes:
There are currently 16 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

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/222/15

Treatment of glioma

Action: For adoption

Document tabled:
Draft Summary report

Notes:
There are currently 40 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/023/08 Topotecan hydrochloride (liposomal), EMEA/OD/034/08 Gadodiamide (liposomal), 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/050/06 Iodine (131I) anti-tenascin monoclonal antibody 81C6, 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/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

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, EMA/OD/031/10 Glutathione-pegylated liposomal doxorubicin hydrochloride, 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/049/12 Humanised monoclonal antibody against epidermal growth factor receptor, EMA/OD/113/15 Dronabinol and cannabidiol

2.2.6. - EMA/OD/199/15

Treatment of familial periodic paralysis

Action: For adoption

Document tabled:
Draft Summary report

2.2.7. - EMA/OD/207/15

Treatment of non-infectious uveitis

Action: For adoption

Document tabled:
Draft Summary report

Notes:
There are currently 4 designations for this condition: EMA/OD/118/12 Voclosporin, EMA/OD/024/15 3-{[2,3,5,6-tetrafluoro-3'-(trifluoromethoxy)biphenyl-4-yl]carbamoyl}thiophene-2-carboxylic acid , EMA/OD/195/14 Autologous collagen type II-specific regulatory T cells, EMA/OD/320/14 Triamcinolone acetonide

2.2.8. - EMA/OD/203/15

Treatment of cutaneous T-cell lymphoma

Action: For adoption

Document tabled:
Draft Summary report

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

2.2.9. - EMA/OD/176/15

Treatment of acute lymphoblastic leukemia

Action: For adoption

Document tabled:
Draft Summary report

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

2.2.10. - EMA/OD/201/15

Diagnosis of glioma

Action: For adoption

Document tabled:
Draft Summary report

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

2.2.11. - EMA/OD/200/15

Diagnosis of hepatocellular carcinoma

Action: For adoption

Document tabled:

Draft Summary report

2.2.12. - EMA/OD/211/15

Treatment of gastro-entero-pancreatic neuroendocrine tumours

Action: For adoption

Document tabled:
Draft Summary report

Notes:
There are currently 2 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

Designation withdrawn: EMEA/OD/040/07 Everolimus

2.2.13. - EMA/OD/181/15

Treatment of monogenic diabetes

Action: For adoption

Document tabled:
Draft Summary report

2.2.14. - EMA/OD/159/15

Treatment of soft tissue sarcoma

Action: For adoption

Document tabled:
Draft Summary report

Notes:
There are currently 7 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

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-cosahydro-3H-23,27-epoxypyrido[2,1-c][1,4]oxazacyclohentriacontin-3-yl]propyl}-2-methoxy-cyclohexyldimethyl-phosphinate, 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

2.2.15. - EMA/OD/209/15

Treatment of graft rejection following solid organ transplantation

Action: For adoption

Document tabled:
Draft Summary report

2.2.16. - EMA/OD/214/15

Treatment of gastro-entero-pancreatic neuroendocrine tumours

Action: For adoption

Document tabled:
Draft Summary report

Notes:
There are currently 2 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

Designation withdrawn: EMEA/OD/040/07 Everolimus

2.2.17. - EMA/OD/206/15

Treatment of glioma

Action: For adoption

Document tabled:
Draft Summary report

Notes:
There are currently 40 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/023/08 Topotecan hydrochloride (liposomal), EMEA/OD/034/08 Gadodiamide (liposomal), 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/050/06 Iodine (131I) anti-tenascin monoclonal antibody 81C6, 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/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-(triflurомethyl)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

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, EMA/OD/031/10 Glutathione-pegylated liposomal doxorubicin hydrochloride, 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/049/12 Humanised monoclonal antibody against epidermal growth factor receptor, EMA/OD/113/15 Dronabinol and cannabidiol

2.2.18. - EMA/OD/198/15

Treatment of acute myeloid leukaemia

Action: For adoption

Document tabled:
Draft Summary report

Notes:

There are currently 43 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

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

2.2.19. - EMA/OD/238/15

Treatment of soft tissue sarcoma

Action: For adoption

Document tabled:
Draft Summary report

Notes:
There are currently 7 designations for this condition: Please 2.2.14.

2.2.20. - EMA/OD/195/15

Treatment of primary hyperoxaluria type 1

Action: For adoption

Document tabled:
Draft Summary report

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

2.2.21. - EMA/OD/193/15

Treatment of progressive supranuclear palsy

Action: For adoption

Document tabled:
Draft Summary report

Notes:
There are currently 3 designations for this condition: EMA/OD/261/14 N-(3-(4-(3-(diisobutylamino)propyl)piperazin-1-yl)propyl)-1H-benzo[d]imidazol-2-amine disulphate

salt, EMA/OD/044/15 Humanised IgG4 monoclonal antibody against extracellular tau, EMA/OD/076/10 Methylthioninium,

Designations withdrawn: EMEA/OD/129/09 Davunetide, EMEA/OD/074/09 4-benzyl-2-naphtalen-1-yl-1,2,4-thiadiazolidine-3,5-dione,

2.2.22. - EMA/OD/194/15

Treatment of behavioural variant fronto-temporal dementia

Action: For adoption

Document tabled:
Draft Summary report

Notes:
There is currently1 designation for this condition: EMA/OD/063/10 Methylthioninium,

2.2.23. - EMA/OD/179/15

Treatment of pulmonary arterial hypertension

Action: For adoption

Document tabled:
Draft Summary report

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

2.2.24. - EMA/OD/205/15

Treatment of acute myeloid leukaemia

Action: For adoption

Document tabled:
Draft Summary report

Notes:
There are currently 43 designations for this condition: Please see 2.2.18.

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 19-21 January 2016 COMP meeting

2.7. Evaluation on-going

Four 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. Table 6. Evaluation Ongoing.

3. Requests for protocol assistance with significant benefit question

3.1. Ongoing procedures

3.1.1. -

Treatment of Niemann-Pick disease, type C

Action: For adoption

3.1.2. -

Treatment of cytomegalovirus disease in patients with impaired cell mediated immunity

Action: For adoption

3.1.3. -

Treatment of advanced ovarian cancer

Action: For adoption

3.2. Finalised letters

3.2.1. -

Treatment of ovarian cancer

Action: For information

3.2.2. -

Prevention of oral mucositis in head and neck cancer patients undergoing radiation therapy

Action: For information

3.2.3. -

Treatment of amyotrophic lateral sclerosis

Action: For information

3.2.4. -

Treatment of ovarian cancer

Action: For information

3.2.5. -

Treatment of acute myeloid leukaemia

Action: For information

3.3. New requests

3.3.1. -

Diagnosis of gastro-entero-pancreatic neuroendocrine tumours

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. Neofordex - dexamethasone – EMEA/OD/133/09, EU/3/10/745, EMEA/H/C/004071

LABORATOIRES CTRS; Treatment of multiple myeloma

Action: For adoption, Oral explanation to be held on 19 January 2016 at time 14:30

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

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

4.1.2. 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: CHMP negative opinion adopted in December 2015.

4.1.3. 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 information

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

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

4.2.1. - 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 discussion

Documents tabled:
Draft report on review of OMPD

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

Bristol-Myers Squibb; Treatment of multiple myeloma

Action: For discussion

Documents tabled:
Draft report on review of OMPD

4.2.3. - 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 discussion

Documents tabled:
Draft report on review of OMPD

4.2.4. - allogeneic T cells genetically modified to express suicide gene - EMEA/OD/041/03, EU/3/03/168, EMEA/H/C/002801

MolMed SpA; Adjunctive treatment in haematopoietic cell transplantation

Action: For discussion

Documents tabled:
Draft report on review of OMPD

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

CSL Behring GmbH; Treatment of haemophilia B

Action: For information

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

Amicus Therapeutics UK Ltd; Treatment of Fabry disease

Action: For information

Notes:
Status of the procedure at the CHMP: CHMP Day 150 LoOI adopted in December 2015.

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

NPS Pharma Holdings Limited; Treatment of hypoparathyroidism

Action: For information

4.2.8. Translarna - ataluren – Type II variation - EMEA/OD/107/04, EU/3/05/277, EMEA/H/C/002720/II/0012

PTC Therapeutics International Limited; Treatment of cystic fibrosis

Action: For information

Notes:
Status of the procedure at the CHMP: CHMP Request for Supplementary Information adopted in December 2015.

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

None

5.2. Coordination with EMA Scientific Committees or CMDh-v

5.2.1. Paediatric Committee (PDCO)

Report from the COMP/PDCO Working Group meeting held in December 2015

Action: For information

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

5.3.1. Significant Benefit Working Group

Proposed meeting time on 21 January 2016 at time 12:00, room 2F

5.4. Cooperation within the EU regulatory network

5.4.1. European Commission

Report on the Commission Expert Group on Rare Diseases meeting held on 12-13 November 2015

Action: For information

Notes: To be presented by Bruno Sepodes (Postponed from December 2015 COMP meeting)

Documents tabled:
Draft agenda - EC Expert Group on Rare Diseases 12-13 November meeting
Draft minutes - 5th EC Expert Group on Rare Diseases meeting

5.5. Cooperation with International Regulators

None

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

None

5.7. COMP work plan

5.7.1. Draft COMP Work Plan 2016

Action: For adoption

Document tabled:
Draft COMP Work Plan 2016

Notes: To be presented by Bruno Sepodes

5.8. Planning and reporting

5.8.1. List of all applications submitted/expected and the COMP coordinatorship distribution of valid applications submitted in 2015-2016

Action: For information

5.8.2. Overview of orphan marketing authorisations/applications

Action: For information

5.8.3. Report on the 'Data Gathering Initiative'

Action: For information

6. Any other business

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

6.1.1. –

Action: For information / appointment of coordinators