4 November 2015
EMA/COMP/674065/2015
Procedure Management and Committees Support Division

Committee for Orphan Medicinal Products (COMP)

Draft agenda for the meeting on 10-12 November 2015

Chair: Bruno Sepodes – Vice-Chair: Lesley Greene

10 November 2015, 09:00-19:30, room 3E

11 November 2015, 08:30-19:30, room 3E

12 November 2015, 08:30-18:00, 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).

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 10-12 November 2015. See November 2015 COMP minutes (to be published post December 2015 COMP meeting).

1.2. Adoption of agenda

COMP agenda for 10-12 November 2015

1.3. Adoption of the minutes

COMP minutes for 6-8 October 2015

2. Applications for orphan medicinal product designation

2.1. For opinion

2.1.1. - EMA/OD/142/15

Treatment of beta-thalassemia intermedia and major

Action: For adoption, Oral explanation to be held on 10 November 2015 at time 09:30

Documents tabled:
Draft Summary report with response to LoQs

Notes:
There have been 2 designations for this condition: EMA/OD/146/12 Autologous CD34+ haematopoietic stem cells transduced with lentiviral vector encoding the human βA-T87Q-globin gene, EMA/OD/189/14 Benserazide hydrochloride

2.1.2. - EMA/OD/138/15

Treatment of cystic fibrosis

Action: For adoption, Oral explanation to be held on 10 November 2015 at time 15:30

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

Notes:
There have been 38 designations for this condition: EMEA/OD/032/00 L-Lysine-N-Acetyl-L-Cysteinate, EMEA/OD/011/03 Recombinant dog gastric lipase, EMEA/OD/038/02 Duramycin, 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, EMA/OD/068/15 Fixed-dose combination of fosfomycin disodium and tobramycin, EMA/OD/061/15 Recombinant human acid ceramidase

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

2.1.3. - EMA/OD/133/15

Treatment of pancreatic cancer

Action: For adoption, Oral explanation to be held on 10 November 2015 at time 17:00

Documents tabled:
Draft Summary report with response to LoQs

Notes:
There have been 32 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

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

2.1.4. - EMA/OD/140/15

Treatment of Duchenne muscular dystrophy

Action: For information

Documents tabled:
Withdrawal request of 20 October 2015

Notes:
Withdrawn
There have been 23 designations for this condition: EMA/OD/142/11 Exon 45 specific phosphorothioate oligonucleotide, EMA/OD/143/11 Exon 53 specific phosphorothioate oligonucleotide, EMEA/OD/106/04 3-[5-(2-fluoro-phenyl)-[1,2,4]oxadiazole-3-yl]-benzoic acid, EMEA/OD/026/05 Adeno-associated viral vector containing a modified U7 snRNA gene, EMEA/OD/077/06 Idebenone, EMEA/OD/065/08 5-(ethylsulfonyl)-2-(naphthalen-2-yl)benzo[d]oxazole, EMEA/OD/049/08 RNA, [P-deoxy-P-(dimethylamino)] (2',3'-dideoxy-2',3'-imino-2',3'-seco) (2'a→5') (C-m5U-C-C-A-A-C-A-m5U-C-A-A-G-G-A-A-G-A-m5U-G-G-

C-A-m5U-m5U-m5U-C-m5U-A-G), P-[4-[[2-[2-(2-hydroxyethoxy)ethoxy]ethoxy]carbonyl]-1-piperazinyl] N,N-dimethylamdinator_Application.Appl, EMEA/OD/081/08 Exon 44 specific phosphorothioate oligonucleotide, EMEA/OD/082/08 Exon 51 specific phosphorothioate oligonucleotide, EMEA/OD/044/09 Adeno-associated viral vector containing modified U1 snRNA, EMEA/OD/083/09 RNA, [P-deoxy-P-(dimethylamino)] (2',3'-dideoxy-2',3'-imino-2',3'-seco) (2'a→5') (C-m5U-m5U-A-C-A-G-G-C-m5U-C-C-A-A-m5U-A-G-m5U-G-G-m5U-C-A-G-m5U), 5' [P-[4-[[2-[2-(2-hydroxyethoxy)ethoxy]ethoxy]carbonyl]-1-piperazinyl]-N,N-dimethylaminophodinator_EMA/OD/090/13 Naproxcinod, EMA/OD/162/11 Halofuginone hydrobromide, EMA/OD/028/12 Givinostat, EMA/OD/121/12 Exon 52 specific phosphorothioate oligonucleotide, EMA/OD/122/12 Exon 55 specific phosphorothioate oligonucleotide, EMA/OD/164/12 Humanised monoclonal antibody against myostatin, EMA/OD/183/12 R,S-O-(3-piperidino-2-hydroxy-1-propyl)-nicotinic acid amidoxime dihydrochloride, EMA/OD/162/13 Asp-Arg-Val-Tyr-Ile-His-Pro, EMA/OD/049/14 17α,21-dihydroxy-16α-methyl-pregna-1,4,9(11)-triene-3,20-dione, EMA/OD/166/14 Adeno-associated viral vector serotype 8 containing the human MD1 gene, EMA/OD/307/14 Rimeporide, EMA/OD/041/15 Allogeneic human adult stem cells, isolated from skeletal muscle and expanded ex vivo

Designations withdrawn: EMEA/OD/096/05 2'-O-methyl-phosphorothioate oligonucleotide, EMEA/OD/025/06 2-(4-(diethylamino) phenyl)-6-methyl-2H-benzo[d][1,2,3] triazol-5-amine, EMA/OD/085/10 Recombinant fusion protein consisting of the extracellular portion of human activin receptor IIB linked to the human IgG1 Fc domain

2.1.5. - EMA/OD/127/15

Treatment of amyotrophic lateral sclerosis

Action: For adoption, Oral explanation to be held on 10 November 2015 at time 18:00

Documents tabled:
Draft Summary report with response to LoQs

Notes:
There have been 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

2.1.6. - EMA/OD/139/15

Treatment of primary sclerosing cholangitis

Action: For adoption, Oral explanation to be held on 11 November 2015 at time 10:00

Documents tabled:
Draft Summary report with response to LoQs

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

2.1.7. - EMA/OD/099/15

Treatment of gastric cancer

Action: For information

Documents tabled:
Withdrawal request of 16 October 2015

Notes:
Withdrawn
There have been 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

2.1.8. - EMA/OD/094/15

Treatment of Primary Sjogren's syndrome

Action: For adoption, Oral explanation to be held on 11 November 2015 at time 12:00

Documents tabled:
Draft Summary report with response to LoQs

2.1.9. - EMA/OD/128/15

Treatment of activated PI3Kdelta syndrome (APDS); p110delta-activating mutation causing senescent T Cells, lymphadenopathy and immunodeficiency (PASLI)

Action: For adoption, Oral explanation to be held on 11 November 2015 at time 14:30

Documents tabled:
Draft Summary report with response to LoQs

2.1.10. - EMA/OD/106/15

Treatment of ascites

Action: For adoption, Oral explanation to be held on 11 November 2015 at time 15:30

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

2.1.11. - EMA/OD/147/15

Treatment of gastric neuroendocrine tumours

Action: For adoption, Oral explanation to be held on 11 November 2015 at time 17:00

Documents tabled:
Draft Summary report with response to LoQs

2.1.12. - EMA/OD/263/14

Treatment of myotonic dystrophy

Action: For adoption, Oral explanation to be held on 11 November 2015 at time 18:00

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

2.1.13. - EMA/OD/125/15

Prevention of mercury toxicity

Action: For adoption, Oral explanation to be held on 12 November 2015 at time 09:00

Documents tabled:
Draft Summary report with response to LoQs

Notes:
There are currently 2 designations for this condition: EMA/OD/119/12 Erdosteine, EMA/OD/093/11 N,N'-bis(2-mercaptoethyl)isophthalamide

2.1.14. - EMA/OD/154/14

Treatment of Wilson's disease

Action: For adoption, Oral explanation to be held on 12 November 2015 at time 09:00

Documents tabled:
Draft Summary report with response to LoQs

Notes:
There have been 4 designations for this condition: EMEA/OD/043/03 Trientine dihydrochloride, EMEA/OD/114/07 Ammonium tetrathiomolybdate, EMA/OD/142/12 Choline tetrathiomolybdate, EMA/OD/001/15 Trientine tetrahydrochloride

2.1.15. - EMA/OD/131/15

Prevention of graft-versus-host disease

Action: For adoption, Oral explanation to be held on 12 November 2015 at time 10:00

Documents tabled:
Draft Summary report with response to LoQs

Notes:
There have been 5 designations for this condition: EMEA/OD/054/06 Recombinant fusion protein consisting of the extracellular portion of CD95 fused to the Fc part of a human IgG1 molecule, EMEA/OD/121/07 Donor lymphocyte preparation depleted of functional alloreactive T-cells, EMA/OD/103/13 Defibrotide, EMA/OD/146/13 Allogeneic bone-marrow derived ex-vivo expanded multipotent adult progenitor cells, EMA/OD/163/14 Allogeneic bone marrow derived mesenchymal cells expanded ex vivo in synthetic media

2.1.16. - EMA/OD/143/15

Treatment of neurotrophic keratitis

Action: For adoption, Oral explanation to be held on 12 November 2015 at time 11:00

Documents tabled:
Draft Summary report with response to LoQs

Notes:
There has been 1 designation for this condition: EMA/OD/185/14 (1S,4R,5R,7S)-3,4-dibenzyl-2-oxo-6,8-dioxa-3-azabyciclo[3.2.1]octane-7-carboxylic acid-L-lysine

2.1.17. - EMA/OD/144/15

Treatment of acute myeloid leukaemia

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

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

Notes:
There have been 42 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/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, 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

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 Ig-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

2.1.18. - EMA/OD/095/15

Treatment of short bowel syndrome

Action: For adoption, Oral explanation to be held on 12 November 2015 at time 14:30

Documents tabled:

Draft Summary report with response to LoQs

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

2.1.19. - EMA/OD/137/15

Treatment of adrenal insufficiency

Action: For adoption, Oral explanation to be held on 12 November 2015 at time 15:30

Documents tabled:
Draft Summary report with response to LoQs

Notes:
There have been 3 designations for this condition: EMEA/OD/009/03 Prasterone, EMEA/OD/108/05 Hydrocortisone (modified release tablet), EMEA/OD/095/06 Hydrocortisone (modified release tablet)

2.2. For discussion / preparation for an opinion

2.2.1. - EMA/OD/153/15

Treatment of pseudohypoaldosteronism type 1B

Action: For adoption

Documents tabled:
Draft Summary report

2.2.2. - EMA/OD/170/15

Treatment of Charcot-Marie-Tooth Disease

Action: For adoption

Documents tabled:
Draft Summary report
COMP coordinator's comments

Notes:
There have been 2 designations for this condition: EMA/OD/193/13 Fixed dose combination of (R-S) baclofen, naltrexone hydrochloride and D-sorbitol, EMEA/OD/100/07 Ascorbic acid

Designations withdrawn: EMEA/OD/164/09 Baclofen/ Naltrexone/ D-Sorbitol,

2.2.3. - EMA/OD/172/15

Treatment of hemophilia B

Action: For adoption

Documents tabled:

Draft Summary report

Notes:
There have been 8 designations for this condition: EMA/OD/041/14 Synthetic double-stranded siRNA oligonucleotide directed against antithrombin mRNA that is covalently linked to a ligand containing three N-acetylgalactosamine residues, EMA/OD/090/11 Adeno-associated viral vector containing the human factor IX gene, EMA/OD/073/14 Recombinant factor VIIa modified with three terminal repeats derived from the β chain of human chorionic gonadotropin, EMEA/OD/062/09 Sequence modified human recombinant factor VIIa, EMEA/OD/117/09 Recombinant fusion protein linking human coagulation factor IX with human albumin, EMA/OD/133/10 Recombinant fusion protein linking human coagulation factor VIIa with human albumin, EMEA/OD/005/09 Pegylated recombinant human factor IX, EMA/OD/003/15 Adeno-associated viral vector containing the human factor IX gene

Designations withdrawn: EMA/OD/099/10 Recombinant human Factor IX, EMA/OD/041/12 Recombinant coagulation factor IX, EMA/OD/151/12 Recombinant human factor VIIa variant, EMEA/OD/008/08 Pegylated recombinant factor VIIa, EMA/OD/070/12 Vatreptacog alfa

2.2.4. - EMA/OD/166/15

Treatment of ovarian cancer

Action: For adoption

Documents tabled:
Draft Summary report

Notes:
There have been 30 designations for this condition: EMEA/OD/019/02 Oregovomab, EMEA/OD/080/03 Anti-epithelial cell adhesion molecule/anti-CD3 monoclonal antibody, EMEA/OD/044/03 Trabectedin, EMA/OD/014/10 Pyr-His-Trp-Ser-Tyr-D-Lys(doxorubicinylglutarate)-Leu-Arg-Pro-Gly-NH2, acetate salt, EMEA/OD/065/05 Imexon, EMEA/OD/061/06 Paclitaxel (micellar), EMEA/OD/063/07 Olaparib, EMEA/OD/110/07 Humanised monoclonal antibody to the folate receptor alpha, EMEA/OD/006/09 Human MHC non-restricted cytotoxic T-cell line, EMEA/OD/086/09 8-[4-(1-aminocyclobutyl)phenyl]-9-phenyl-1,2,4-triazolo[3,4-f][1,6]naphthyridin-3(2H)-one mono-hydrochloride, EMA/OD/015/10 (3S)-3-{4-[7-(aminocarbonyl)-2H-indazol-2-yl] phenyl} piperidine tosylate monohydrate salt, EMA/OD/021/10 Autologous dendritic cells pulsed with recombinant human-fusion protein (mucin 1 - glutathione S transferase) coupled to oxidised polymannose, EMA/OD/111/10 Veliparib, EMA/OD/054/11 20-pentaerythritol poly (oxy-1,2-ethanediyl)-carboxymethyl-glycinate-7-ethyl-10-hydroxycamptothecine 10-[1,4'-bipiperidine]-1'-carboxylate, EMA/OD/094/11 Vincaleukoblastin-23-oic acid, O4-deacetyl-2-[(2-mercaptoethoxy)carbonyl]hydrazide, disulfide with …, EMA/OD/151/11 2-Allyl-1-[6-(1-hydroxy-1-methylethyl)pyridin-2-yl]-6-{[4-(4-methylpiperazin-1-yl)phenyl]amino}-1,2-dihydro-3H-pyrazolo[3,4-d]pyrimidin-3-one, EMA/OD/085/12 rucaparib, EMA/OD/099/12 Lurbinectedin, EMA/OD/114/12 Alisertib, EMA/OD/147/12 Chimeric monoclonal antibody against claudin 6, EMA/OD/039/13 Fosbretabulin tromethamine, EMA/OD/122/13 Trebananib, EMA/OD/186/13 Genetically modified serotype 5/3 adenovirus coding for granulocyte macrophage colony-stimulating factor, EMA/OD/059/14 Cediranib, EMA/OD/281/14 Humanised anti-folate receptor 1 monoclonal antibody conjugated to maytansinoid DM4, EMA/OD/157/14 2-hydroxymethyl-2-methoxymethyl-1-

azabicyclo[2,2,2]octan-3-one, EMA/OD/211/14 Chimeric group B adenovirus (11p/3) with deletions in the E3 and E4 regions, EMA/OD/223/14 N-methyl-4-({4-[({3-methyl(methylsulfonyl)amino]pyrazin-2- yl}methyl)amino]-5- (trifluoromethyl)pyrimidin-2-yl}amino)benzamide hydrochloride, EMA/OD/304/14 Human reovirus type 3 Dearing strain, EMA/OD/314/14 {2-amino-8-[4-(pyrrolidinylcarbonyl)phenyl]-(3H-benzo[f]azepin-4-yl)}-N,N-dipropylcarboxamide

Designations withdrawn: EMEA/OD/061/00 Human Milk Fat Globule 1 / Yttrium (90Y) human Milk Fat Globule 1 - S p isothiocyanatobenzyl-diethylenetriaminepentaacetic acid, EMEA/OD/062/01 Epothilone B, EMEA/OD/016/03 Murine anti-idiotypic antibody against OC125 antibody against CA125 antigen, EMEA/OD/071/09 Anti-EphA2 monoclonal antibody conjugated to maleimidocaproyl monomethylauristatin phenylalanine, EMA/OD/002/12 1-(4-{4-amino-7-[1-(2-hydroxyethyl)-1H- pyrazol-4-yl] thieno [3,2-c]pyridin-3-yl}phenyl)-3-(3-fluorophenyl)urea

2.2.5. - EMA/OD/163/15

Treatment of partial deep dermal and full thickness burns

Action: For adoption

Documents tabled:
Draft Summary report
COMP coordinator's comments

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

2.2.6. - EMA/OD/164/15

Treatment of non-infectious uveitis

Action: For adoption

Documents tabled:
Draft Summary report
COMP coordinator's comments

Notes:
There have been 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

2.2.7. - EMA/OD/149/15

Treatment of monogenic diabetes

Action: For adoption

Documents tabled:
Draft Summary report

2.2.8. - EMA/OD/116/15

Treatment of acute myeloid leukemia

Action: For adoption

Documents tabled:
Draft Summary report

Notes:
There have been 42 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/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, 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

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 Ig-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

2.2.9. - EMA/OD/148/15

Treatment of interstitial lung disease in children

Action: For adoption

Documents tabled:
Draft Summary report

2.2.10. - EMA/OD/155/15

Treatment of myelodysplastic syndromes

Action: For adoption

Documents tabled:
Draft Summary report
COMP coordinator's comments

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

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

2.2.11. - EMA/OD/154/15

Treatment of primary myelofibrosis

Action: For adoption

Documents tabled:
Draft Summary report
COMP coordinator's comments

Notes:
There have been 5 designations for this condition: EMA/OD/019/10 11-(2-pyrrolidin-1-yl-ethoxy)-14,19-dioxa-5,7,26-triaza-tetracyclo[19.3.1.1(2,6).1(8,12)] heptacosa-1(25),2(26),3,5,8,10,12(27),16,21,23-decaene, EMEA/OD/161/09 Pomalidomide, EMA/OD/069/10 N-tert-butyl-3-[(5-methyl-2-{[4-(2-pyrrolidin-1-ylethoxy)phenyl]amino}pyrimidin-4-yl)amino] benzenesulfonamide dihydrochloride monohydrate, EMA/OD/152/10 N-(cyanomethyl)-4-(2-{[4-(morpholin-4-yl)phenyl]amino}pyrimidin-4-yl)benzamide, dihydrochloride salt, EMA/OD/140/14 Recombinant human Pentraxin-2

Designation withdrawn: EMA/OD/123/10 Plitidepsin

2.2.12. - EMA/OD/162/15

Treatment of osteosarcoma

Action: For adoption

Documents tabled:
Draft Summary report

Notes:
There have been 3 designations for this condition: EMEA/OD/013/04 Muramyl Tripeptide Phosphatidyl Ethanolamine, EMEA/OD/004/07 5(S)-(2'-hydroxy ethoxy)-20(S)-Camptothecin, EMA/OD/020/13 Lipid-complexed cisplatin

2.2.13. - EMA/OD/168/15

Treatment of pancreatic cancer

Action: For adoption

Documents tabled:
Draft Summary report

Notes:
There have been 32 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

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

2.2.14. - EMA/OD/157/15

Treatment of malignant mesothelioma

Action: For adoption

Documents tabled:
Draft Summary report

Notes:
There have been 8 designations for this condition: EMEA/OD/003/08 NGR-human Tumour Necrosis Factor, EMA/OD/063/12 Maytansinoid-conjugated human monoclonal antibody against mesothelin, EMA/OD/012/13 N-methyl-4-({4-[({3-methyl(methylsulfonyl)aminopyrazin-2-yl}methyl)amino]-5-(trifluoromethyl)pyrimidin-2-yl}amino)benzamide hydrochloride, EMA/OD/138/13 Autologous dendritic cells pulsed with allogeneic tumour cell lysate, EMA/OD/108/13 Amatuximab, EMA/OD/076/14 Pegylated recombinant arginine deiminase, EMA/OD/180/14 Genetically modified serotype 5/3 adenovirus coding for granulocyte macrophage colony-stimulating factor, EMA/OD/168/14 5-[8-methyl-9-(1-methylethyl)-2-(4-morpholinyl)-9H-purin-6-yl]-2-pyrimidinamine

Designations withdrawn: EMEA/OD/022/01 Pemetrexed disodium, EMA/OD/028/10 Vorinostat

2.2.15. - EMA/OD/150/15

Treatment of Merkel cell carcinoma

Action: For adoption

Documents tabled:
Draft Summary report

Notes:
Designation withdrawn: EMEA/OD/155/09 Maytansinoid-conjugated humanised monoclonal antibody against CD56

2.2.16. - EMA/OD/160/15

Treatment of soft tissue sarcoma

Action: For adoption

Documents tabled:
Draft Summary report

Notes:
There have been 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-codinator, EMEA/OD/071/05 Brostallicin, EMEA/OD/083/06 Fenretinide, EMEA/OD/044/08 Palifosfamide, EMA/OD/141/10 Ombrabulin

2.2.17. - EMA/OD/165/15

Treatment of acute myeloid leukaemia

Action: For adoption

Documents tabled:
Draft Summary report

Notes:
There have been 42 designations for this condition: please see 2.2.8.

2.2.18. - EMA/OD/123/15

Treatment of arginase deficiency

Action: For adoption

Documents tabled:
Draft Summary report

Notes:

Designations withdrawn: EMA/OD/231/14 Sodium benzoate

2.2.19. - EMA/OD/124/15

Treatment of argininosuccinate lyase deficiency

Action: For adoption

Documents tabled:
Draft Summary report

Notes:
Designations withdrawn: EMA/OD/230/14 sodium benzoate,

2.2.20. - EMA/OD/151/15

Treatment of pseudohypoaldosteronism type 1B

Action: For adoption

Documents tabled:
Draft Summary report

2.2.21. - EMA/OD/169/15

Treatment of pancreatic cancer

Action: For adoption

Documents tabled:
Draft Summary report

Notes:
There have been 32 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

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

2.2.22. - EMA/OD/122/15

Treatment of post cardiac arrest syndrome

Action: For adoption

Documents tabled:
Draft Summary report

2.2.23. - EMA/OD/152/15

Treatment of pulmonary arterial hypertension

Action: For adoption

Documents tabled:
Draft Summary report
COMP coordinator's comments

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

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 10-12 November 2015 COMP meeting

2.7. Evaluation on-going

Eighteen 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 ovarian cancer

Action: For adoption

3.1.2. -

Treatment of amyotrophic lateral sclerosis

Action: For adoption

3.1.3. -

Treatment of glycogen storage disease type II (Pompe's disease)

Action: For adoption

3.1.4. -

Treatment of growth hormone deficiency

Action: For adoption

3.1.5. -

Treatment of Prader-Willi syndrome

Action: For adoption

3.2. Finalised letters

3.2.1. -

Treatment of systemic sclerosis

Action: For information

3.2.2. -

Treatment of acromegaly

Action: For information

3.2.3. -

Treatment of sickle cell disease

Action: For information

3.3. New requests

3.3.1. -

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

Action: For information

3.3.2. -

Treatment of Niemann-Pick disease, type C

Action: For information

3.3.3. -

Treatment of cytomegalovirus disease in patients with impaired cell mediated immunity

Action: For information

3.3.4. -

Treatment of advanced ovarian cancer

Action: For information

3.3.5. -

Treatment of acute myeloid leukaemia

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. Heparesc - human heterologous liver cells - 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

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

Notes:
Status of the procedure at the CHMP: Re-examination of initial application: CAT and CHMP adopted **negative** opinion in October 2015.

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

4.2.1. - Dexamethasone acetate – EMEA/H/C/004071

LABORATOIRES CTRS; Treatment of multiple myeloma

Action: For discussion

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

4.2.2. - recombinant l-asparaginase – EMA/OD/063/04, EU/3/04/258, 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

4.2.3. 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 discussion

Document tabled:
Draft report on review of OMPD

4.2.4. - 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 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

Joint COMP- PDCO Joint Strategic Review & Learning Meeting held in Bonn on 14-16 October 2015

Action: For information

Strategic Review & Learning Meetings organised during the term of the European Presidency:
- Organisational aspects
- Clarification on responsibility for handling of declared interests and on involvement of external (non NCA) speakers

Documents tabled:
Principles for organisation of NCA hosted meetings
Responsibilities for confidentiality in NCA Hosted Meetings

Action: For information

Notes: Postponed to November COMP meeting.

5.2. Coordination with EMA Scientific Committees or CMDh-v

5.2.1. Committee for Medicinal Products for Human Use (CHMP)

Public consultation comments on the CHMP Guideline concerning Conditional Marketing Authorisation

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 12 November 2015 from 17:00-18:00, room 3E

Action: For information

Document tabled:
SBWG meeting documents

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

Work plan for the European Medicines Agency Human Scientific Committees' Working Party with Patients' and Consumers' Organisations (PCWP) 2016

Action: For adoption

Document tabled:
PCWP Work plan 2016
Draft Agenda - PCWP meeting with all eligible organisations 26 November 2015
Draft Agenda - Training session for patients and consumers interested in EMA activities 25 November 2015

5.3.3. Working Party with Healthcare Professionals' Organisations (HCPWP)

Work plan for the European Medicines Agency Human Scientific Committees' Working Party with Healthcare Professionals' Organisations (HCPWP) 2016

Action: For adoption

Document tabled:
HCPWP Work plan 2016

5.4. Cooperation within the EU regulatory network

None

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 discussion

Document tabled:
Draft COMP Work Plan 2016

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

6. Any other business

None