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SCIENCE MEDICINES HEALTH

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Inspections, Human Medicines Pharmacovigilance and Committees Division

Committee for Orphan Medicinal Products (COMP)

Draft agenda for the meeting on 11-13 September 2018

Chair: Bruno Sepodes

11 September 2018, 08:30-19:30, room 02-F

12 September 2018, 08:30-19:30, room 02-F

13 September 2018, 08:30-18:00, room 02-F

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 11-13 September 2018. See September 2018 COMP minutes (to be published post October 2018 COMP meeting).

1.2. Adoption of agenda

COMP agenda for 11-13 September 2018.

1.3. Adoption of the minutes

COMP minutes for 17-19 July 2018.

2. Applications for orphan medicinal product designation

2.1. For opinion

2.1.1. - EMA/OD/074/18

Treatment of polycythemia vera

Action: For information

Document(s) tabled:

Withdrawal request of 7 September 2018

2.1.2. - EMA/OD/089/18

Treatment of neuronal ceroid lipofuscinosis

Action: For information

Document(s) tabled:

Withdrawal request of 17 August 2018

Notes: There have been 3 designations for this condition: EMA/OD/195/16 Recombinant self-complementary adeno-associated viral vector serotype 9 encoding the human CLN3gene, EMA/OD/218/17 Gemfibrozil, EMA/OD/013/18 Adeno-associated viral vector serotype 9 containing the human CLN1 gene

2.1.3. - EMA/OD/094/18

Treatment of acute myeloid leukaemia

Action: For adoption, Oral explanation to be held on 11 September 2018 at 15:00

Document(s) tabled:

Draft Summary report with response to LoQs

Notes: There have been 54 designations for this condition: EMEA/OD/022/00 Gemtuzumab ozogamicin, EMEA/OD/028/04 Midostaurin, EMEA/OD/056/06 Antisense oligonucleotide 5'-d[P-Thio] (CCCTG CTCCC CCCTG GCTCC)-3' (see comments box for cenersen sodium), 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/049/07 5'-O-(trans-9"-octadecenoyl)-1-β-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 dihydrochloride salt, EMEA/OD/028/09 Tosedostat, EMEA/OD/091/09 1-Cyclopropyl-3-[3-(5-morpholin-4-ylmethyl-1H-benzimidazol-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/044/10 Allogeneic T cells encoding an exogenous TK gene, EMA/OD/094/10 N-[(2S)-2,3-dihydroxypropyl]-3-[(2-fluoro-4-iodophenyl) amino] isonicotinamide hydrochloride, 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/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/258/14 Ulocuplumab, 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 pyrrolbenzodiazepine 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, EMA/OD/180/15 Arsenic trioxide, EMA/OD/205/15 Venetoclax, EMA/OD/233/15 Tyr-Met-Phe-Pro-Asn-Ala-Pro-Tyr-Leu, Ser-Gly-Gln-Ala-Tyr-Met-Phe-Pro-Asn-Ala-Pro-Tyr-Leu-Pro-Ser-Cys-Leu-Glu-Ser, Arg-Ser-Asp-Glu-Leu-Val-Arg-His-His-Asn-Met-His-Gln-Arg-Asn-Met-Thr-Lys-Leu and Pro-Gly-Cys-Asn-Lys-Arg-Tyr-Phe-Lys-Leu-Ser-His-Leu-Gln-Met-His-Ser-Arg-Lys-His-Thr-Gly, EMA/OD/253/15 2-methyl-1-[(4-[6-(trifluoromethyl)pyridin-2-yl]-6-{[2-

(trifluoromethyl)pyridin-4-yl]amino}-1,3,5-triazin-2-yl)amino]propan-2-ol methanesulfonate, EMA/OD/155/16 P-ethoxy growth factor receptor-bound protein 2 (Grb2) antisense oligonucleotide, EMA/OD/197/16 Ivosidenib, EMA/OD/319/16 225Ac-lintuzumab, EMA/OD/106/17 Glasdegib maleate, EMA/OD/010/17 Sodium (1R, 3R, 4R, 5S)-3-({2-N-acetylamino-2-deoxy-3-O..., EMA/OD/040/17 Entospletinib, EMA/OD/101/17 Pracinostat, EMA/OD/175/17 Gilteritinib, EMA/OD/193/17 6-{{(1R,2S)-2-aminocyclohexyl}amino}-7-fluoro-4-(1-methyl-1H-pyrazol-4-yl)-1,2-dihydro-3H-pyrrolo[3,4-c]pyridin-3-one monocitrate

Designations withdrawn: EMEA/OD/065/02 2-chloro-9-[2-deoxy-2-fluoro-β-D-arabinofuranosyl]adenine, EMA/OD/051/04 Homoharringtonine, EMA/OD/059/04 Val-Leu-Gln-Glu-Leu-Asn-Val-Thr-Val (Pr1 nanopeptide, sequence 169-177, of proteinase 3), EMA/OD/045/05 Troxacitabine, EMA/OD/018/06 Human monoclonal antibody against inhibitory killer cell Ig-like receptors (1-7 F9), EMA/OD/020/06 Lestaurtinib, EMA/OD/024/07 Arsenic trioxide, EMA/OD/069/07 Amonafide L-malate, EMA/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, EMA/OD/118/08 Lintuzumab, EMA/OD/090/08 Allogeneic ex vivo expanded umbilical cord blood cells, EMA/OD/016/09 26 base single stranded phosphodiester DNA oligonucleotide, EMA/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/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/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, 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

2.1.4. - EMA/OD/081/18

Prevention of graft-versus-host disease

Action: For information

Document(s) tabled:

Withdrawal request of 21 August 2018

Notes: There have been 6 designations for this condition: EMA/OD/054/06 Recombinant fusion protein consisting of the extracellular portion of CD95 fused to the Fc part of a human IgG1 molecule, EMA/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, EMA/OD/119/15 Humanised fusion protein consisting of extracellular domain of CD24 linked to IgG1 Fc domain

Designation withdrawn: EMA/OD/131/15 2-amino-2-[2-[2-chloro-4-[[3-(phenylmethoxy)phenyl]thio]phenyl]ethyl]-1,3-propanediol hydrochloride

2.1.5. - EMA/OD/090/18

Treatment of Fanconi anemia

Action: For adoption, Oral explanation to be held on 12 September 2018 at 11:00

Document(s) tabled:

Draft Summary report with response to LoQs

2.1.6. - EMA/OD/042/18

Treatment of osteosarcoma

Action: For information

Document(s) tabled:

Withdrawal request of 26 July 2018

Notes: There have been 3 designations for this condition: EMEA/OD/013/04 Muramyl Tripeptide Phosphatidyl Ethanolamine, EMA/OD/020/13 Lipid-complexed cisplatin, EMA/OD/162/15 Live attenuated *Listeria monocytogenes* bioengineered with a chimeric human epidermal growth factor receptor 2 fused to a truncated form of the Lm protein listeriolysin O

Designation withdrawn: EMEA/OD/004/07 5(S)-(2'-hydroxy ethoxy)-20(S)-Camptothecin

2.1.7. - EMA/OD/078/18

Treatment of acute liver failure

Action: For adoption, Oral explanation to be held on 12 September 2018 at 14:00

Document(s) tabled:

Draft Summary report with response to LoQs

Notes: There have been 8 designations for this condition: EMEA/OD/037/05 Human heterologous liver cells (for infusion), EMEA/OD/085/08 Recombinant human hepatocarcinoma-intestine-pancreas / pancreatic associated protein, EMA/OD/030/11 Cardiotrophin-1, EMA/OD/105/11 Ornithine phenylacetate, EMA/OD/153/11 Heterologous human adult liver-derived stem cells, EMA/OD/032/13 Immortalised human C3A hepatoblastoma cells, EMA/OD/022/16 Citric acid monohydrate, EMA/OD/222/16 Human hepatoma cell line HepaRG in bioartificial liver

2.1.8. - EMA/OD/077/18

Treatment of acute radiation syndrome

Action: For adoption, Oral explanation to be held on 12 September 2018 at 16:00

Document(s) tabled:

Draft Summary report with response to LoQs

Notes: There have been 3 designations for this condition: EMA/OD/060/15 Fibrinogen-coated albumin spheres, EMA/OD/191/15 Entolimod, EMA/OD/116/16 Recombinant human interleukin-12

2.1.9. - EMA/OD/091/18

Treatment of multiple myeloma

Action: For adoption, Oral explanation to be held on 12 September 2018 at 17:00

Document(s) tabled:

Draft Summary report with response to LoQs

Notes: There have been 16 designations for this condition: EMEA/OD/040/01 Thalidomide, EMEA/OD/044/04 Aplidine, EMEA/OD/066/04 Recombinant histidine-tagged idiotype immunoglobulin Fab fragment of clonal B-cell receptors, EMEA/OD/012/05 N-(methyl-diazacyclohexyl-methylbenzamide)-azaphenyl-aminothiopyrrole, EMEA/OD/120/07 Carfilzomib, EMEA/OD/068/08 N2'-Deacetyl-N2'-[4-methyl-4-(oxobuthyldithio)-1-oxopentyl]-maytansine-chimerized anti-CD138 IgG4 monoclonal antibody, EMEA/OD/076/08 Human anti-intercellular adhesion molecule-1 monoclonal antibody, EMEA/OD/053/08 Milatuzumab, EMEA/OD/053/09 Pomalidomide, EMA/OD/017/11 Acadesine, EMA/OD/048/11 2,2'-{2-[(1R)-1-({[(2,5-dichlorobenzoyl)amino]acetyl)amino]-3-methylbutyl]-5-oxo-1,3,2-dioxaborolane-4,4-diyl}diacetic acid, EMA/OD/113/12 Panobinostat, EMA/OD/125/17 Autologous ex-vivo-expanded peripheral polyclonal lymphocytes enriched in activated natural killer cells, EMA/OD/121/16 Venetoclax, EMA/OD/270/16 Autologous T lymphocyte-enriched population of cells transduced with a lentiviral vector encoding a chimeric antigen receptor targeting human B cell maturation antigen with 4-1BB and CD3-zeta intracellular signalling domains, EMA/OD/077/17 Humanised monoclonal antibody targeting B-cell maturation antigen conjugated with maleimidocaproyl monomethyl auristatin F
Designations withdrawn: EMEA/OD/048/00 Arsenic trioxide, EMEA/OD/003/01 Humanised anti-HM1.24 monoclonal antibody, EMEA/OD/018/00 Thalidomide, EMEA/OD/026/01 Deoxyribose phosphorothioate (5'-tct-ccc-agg-gtg-cgc-cat-3'), EMEA/OD/019/01 Thalidomide, EMEA/OD/070/04 17-allylamino-17-demethoxygeldanamycin, EMEA/OD/093/05 Human monoclonal antibody against HLA-DR, EMEA/OD/003/09 Chimeric anti-interleukin-6 monoclonal antibody, EMEA/OD/133/09 Dexamethasone (40 mg tablet), EMEA/OD/130/09 Perifosine, EMA/OD/115/10 Maytansinoid-conjugated humanised monoclonal antibody against CD56, EMA/OD/137/10 Vorinostat, EMA/OD/137/11 Chimeric monoclonal antibody against kappa myeloma antigen, EMA/OD/061/12 Elotuzumab

2.1.10. - EMA/OD/087/18

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

Action: For adoption, Oral explanation to be held on 12 September 2018 at 15:00

Document(s) tabled:

Draft Summary report with response to LoQs

2.1.11. - EMA/OD/079/18

Treatment of mastocytosis

Action: For adoption, Oral explanation to be held on 13 September 2018 at 10:00

Document(s) tabled:

Draft Summary report with response to LoQs

Notes: There have been 5 designations for this condition: EMEA/OD/062/04 N-(methyl-diazacyclohexyl-methylbenzamide)-azaphenyl-aminothiopyrrole, EMA/OD/016/10 Midostaurin, EMA/OD/075/14 Recombinant human diamine oxidase, EMA/OD/079/13 Cladribine, EMA/OD/087/17 Recombinant monoclonal antibody to sialic acid-binding Ig-like lectin 8

2.2. For discussion / preparation for an opinion

2.2.1. - EMA/OD/114/18

Treatment of dermatomyositis

Action: For discussion/adoption

Document(s) tabled:

Draft Summary report

Notes: Designations withdrawn: EMEA/OD/038/03 Human immunoglobulin, EMA/OD/146/14 Siponimod

2.2.2. - EMA/OD/096/18

Treatment of ATTR amyloidosis

Action: For discussion/adoption

Document(s) tabled:

Draft Summary report

Notes: There has been 1 designation for this condition: EMA/OD/098/13 Phosphorothioate oligonucleotide targeted to transthyretin

Designation withdrawn: EMA/OD/194/13 Synthetic double-stranded siRNA oligonucleotide directed against transthyretin mRNA and covalently linked to a ligand containing three N-acetylgalactosamine residues

2.2.3. - EMA/OD/040/18

Treatment of Kabuki syndrome

Action: For discussion/adoption

Document(s) tabled:

Draft Summary report

2.2.4. - EMA/OD/125/18

Treatment of medullary thyroid cancer

Action: For discussion/adoption

Document(s) tabled:

Draft Summary report

Treatment of cystic fibrosis

Action: For discussion/adoption

Document(s) tabled:

Draft Summary report

Notes: There have been 40 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, EMEA/OD/039/04 Dexamethasone sodium phosphate encapsulated in human erythrocytes, EMEA/OD/053/04 Alpha-1 antitrypsin (inhalation use), EMEA/OD/062/05 Mannitolum, 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, phosphatidylinositol and cholesterol, EMA/OD/046/11 Cysteamine, EMA/OD/058/12 Alpha-1 proteinase inhibitor (for inhalation use), EMA/OD/005/13 Recombinant human CXCL8 mutant, EMA/OD/017/13 4,6,4'-trymethylangelicin, EMA/OD/096/13 Antisense oligonucleotide targeting the F508delta mutation of CFTR, EMA/OD/095/13 Nitric oxide, EMA/OD/159/13 Cysteamine, EMA/OD/156/13 11-(4-Dimethylamino-3-hydroxy-6-methyl-tetrahydro-pyran-2-yloxy)-2-ethyl-3,4,10-trihydroxy-3,5,6,8,10,12,14-heptamethyl-1-oxa-6-aza-cyclopentadecane-13,15-dione, EMA/OD/036/14 Nitric oxide, EMA/OD/013/14 Plasmid DNA encoding the human cystic fibrosis transmembrane conductance regulator gene complexed with a non-viral, cationic lipid based gene transfer agent, EMA/OD/002/14 1-(2,2-difluoro-1,3-benzodioxol-5-yl)-N-{1-[(2R)-2,3-dihydroxypropyl]-6-fluoro-2-(1-hydroxy-2-methylpropan-2-yl)-1H-indol-5-yl}cyclopropanecarboxamide, EMA/OD/131/14 4-[[[(1S,4S)-5-[[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, EMA/OD/013/16 Sodium nitrite and ethylenediaminetetraacetic acid, EMA/OD/156/16 1-(2,2-difluoro-2H-1,3-benzodioxol-5-yl)-N-{1-[(2R)-2,3-dihydroxypropyl]-6-fluoro-2-(1-hydroxy-2-methylpropan-2-yl)-1H-indol-5-yl}cyclopropane-1-carboxamide and ivacaftor, EMA/OD/100/16 (6aR, 10aR)-3-(1',1'-dimethylheptyl)-delta-8-tetrahydro-cannabinol-9-carboxylic acid, EMA/OD/203/17 (R)-2-(5-cyano-2-(6-(methoxycarbonyl)-7-methyl-3-oxo-8-(3-(trifluoromethyl)phenyl)-2,3,5,8-tetrahydro-[1,2,4]triazolo[4,3-a]pyrimidine-5-yl)phenyl)-N,N,N-trimethylethanaminium methanesulfonate dehydrate, EMA/OD/303/16 Phosphoinositide 3-kinase gamma peptide, EMA/OD/006/17 Tamoxifen citrate, EMA/OD/085/17 Teicoplanin

Designations withdrawn: EMEA/OD/009/02 Carbamic acid /[[4-[[3-[[4-[1-(4-hydroxyphenyl)-1-methyl-ethyl]phenoxy]methyl]phenyl]methoxy]-phenyl]iminomethyl]-ethyl ester, EMEA/OD/064/00 8-cyclopentyl-1, 3-dipropylxanthine, EMEA/OD/018/03

Engineered protein inhibitor of human neutrophil elastase, EMEA/OD/075/02 Amiloride hydrochloride dihydrate, EMEA/OD/023/04 Recombinant human bile salt-stimulated lipase, EMEA/OD/107/04 3-[5-(2-fluoro-phenyl)-[1,2,4]oxadiazole-3-yl]-benzoic acid, EMEA/OD/054/05 Heparin sodium (inhalation use), EMEA/OD/072/05 Denufosol tetrasodium, EMEA/OD/118/05 Glutathione, EMEA/OD/024/08 Levofloxacin hemihydrate, EMA/OD/024/10 3-(6-(1-(2,2-difluorobenzo [d] [1,3] dioxol-5-yl)cyclopropanecarboxamido)-3-methylpyridin-2-yl)benzoic acid, EMA/OD/032/14 Lumacaftor/ivacaftor

2.2.6. - EMA/OD/258/17

Treatment of ovarian cancer

Action: For discussion/adoption

Document(s) tabled:

Draft Summary report

Notes: There have been 30 designations for this condition: EMEA/OD/019/02 Oregovomab, EMEA/OD/061/06 Paclitaxel (micellar), EMEA/OD/080/03 Anti-epithelial cell adhesion molecule/anti-CD3 monoclonal antibody, EMEA/OD/044/03 Trabectedin, EMEA/OD/065/05 Imexon, 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-hydroxycamptothecin 10-[1,4'-bipiperidine]-1'-carboxylate, 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/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/126/15 (5S,8S,10aR)-N-benzhydryl-5-((S)-2-(methylamino)propanamido)-3-(3-methylbutanoyl)-6-oxodecahydropyrrolo[1,2-a][1,5]diazocine-8-carboxamide, EMA/OD/159/16 Vaccine consisting of 5 survivin peptides with different human leukocyte antigen restrictions, EMA/OD/300/16 Poly-cyclodextrin-bis-cysteine-PEG3400-camptothecin-conjugate, EMA/OD/035/17 Ofranergene obadenovec, EMA/OD/246/17 Autologous dendritic cells pulsed with killed ovarian cancer cells and matured by TLR3 ligand ex vivo

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 Epophilone B, EMEA/OD/016/03 Murine anti-idiotypic antibody against

OC125 antibody against CA125 antigen, EMEA/OD/063/07 Olaparib, EMEA/OD/071/09 Anti-EphA2 monoclonal antibody conjugated to maleimidocaproyl monomethylauristatin phenylalanine, EMA/OD/014/10 Pyr-His-Trp-Ser-Tyr-D-Lys(doxorubicinylglutarate)-Leu-Arg-Pro-Gly-NH₂, acetate salt, EMA/OD/094/11 Vincalukoblastin-23-oic acid, O₄-deacetyl-2-[(2-mercaptoethoxy)carbonyl]hydrazide, disulfide with ..., 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, EMA/OD/114/12 Alisertib, EMA/OD/314/14 {2-amino-8-[4-(pyrrolidinylcarbonyl)phenyl]-(3H-benzo[f]azepin-4-yl)}-N,N-dipropylcarboxamide

2.2.7. - EMA/OD/113/18

Treatment of patients affected by Mucopolysaccharidosis Type I, Hurler variant (MPSI-H)

Action: For discussion/adoption

Document(s) tabled:

Draft Summary report

2.2.8. - EMA/OD/110/18

Treatment of pancreatic cancer

Action: For discussion/adoption

Document(s) tabled:

Draft Summary report

Notes: There have been 36 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/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-GNNDENISFKK, 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/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/071/13 Chimeric monoclonal antibody against claudin-18 splice variant 2, EMA/OD/164/13 Cysteamine bitartrate, EMA/OD/081/14 Immunoglobulin G1, anti-(human tumour-associated calcium signal transducer 2)(human-Mus musculus monoclonal hRS7 heavy chain), disulfide with human-Mus musculus monoclonal hRS7 κ -chain, dimer, hexakis(thioether) with (4S)-4-[[[4-[(2S)-2-(4-aminobutyl)-2-[[2-[[2-[[26-[4-[[[4-[(3-mercapto-2,5-dioxo-1-pyrrolidinyl)methyl]cyclohexyl]carbonyl]amino]methyl]-1H-1,2,3-triazol-1-yl]-3,6,9,12,15,18,21,24-octaoxahexacos-1-yl]amino]-2-oxoethoxy]acetyl]amino]-1-oxoethyl]amino]phenyl]methoxy]carbonyl]oxy]-4,11-diethyl-9-hydroxy-1H-pyrano[3',4':6,7]indolizino[1,2-b]quinoline-3,14(4H,12H)-dione, 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 inIB strain expressing human mesothelin, EMA/OD/169/15 Two allogenic irradiated pancreatic tumour cell lines, EMA/OD/193/16 Pegylated recombinant human interleukin-10, EMA/OD/241/16 Antroquinonol, EMA/OD/273/16 Humanised IgG4 monoclonal antibody to the human toll-like receptor type 2, EMA/OD/078/17 Sodium 2-hydroxylinoleate, EMA/OD/111/17 Adenoviral vector of serotype 5 modified to contain a chimeric sequence consisting of a minimal urokinase-type plasminogen activator receptor promoter preceded by three Notch-responsive elements, and coated with oligopeptide end-modified poly (beta-amino) esters, EMA/OD/118/17 4-amino-1-[(1S,4R,5S)-2-fluoro-4,5-dihydroxy-3-(hydroxymethyl)cyclopent-2-en-1-yl]pyrimidin-2-one

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/111/07 Chimeric antibody to mesothelin, 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/150/10 Salirasib, EMA/OD/007/12 Polyinosine-polycytidylic acid coupled with the polycationic polyethyleneimine, 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/145/12 Recombinant human monoclonal antibody of the IgG1 kappa class against prostate stem cell antigen, EMA/OD/037/13 (1-methyl-2-nitro-1H-imidazole-5-yl)methyl N,N'-bis(2-bromoethyl)diamidophosphate

2.2.9. - EMA/OD/086/18

Treatment of hepatocellular carcinoma

Action: For discussion/adoption

Document(s) tabled:

Draft Summary report

Notes: There have been 19 designations for this condition: EMEA/OD/015/02 Thymalfasin, EMEA/OD/087/04 Pegylated arginine deiminase, EMEA/OD/048/04 Doxorubicine polyisohexylcyanoacrylate nanoparticles, EMEA/OD/018/05 Nemorubicin hydrochloride, EMEA/OD/070/09 NGR-human tumour necrosis factor, EMEA/OD/076/09 Vaccinia GM-CSF/TK-deactivated virus, EMA/OD/065/10 (S)-10-[(dimethylamino)methyl]-4-ethyl-9-hydroxy-4-O-[alpha-(2", 4", 5", 7"-tetranitro-9"-fluorenylideneaminoxy)propionyl]-1H-pyrano[3', 4', 6', 7']indolizino[1,2-beta]-quinoline-3, 14-(4H, 12H)-dione, hydrochloride, EMA/OD/096/10 Doxorubicin hydrochloride (in heat-sensitive liposomes), EMA/OD/170/10 Sulfonated monophosphorylated mannose oligosaccharide, EMA/OD/003/11 Peretinoin, EMA/OD/045/11 Resminostat, EMA/OD/159/12 4-[2-(6-methylpyridin-2-yl)-5,6-dihydro-4H-pyrrolo[1,2-b]pyrazol-3-yl]-quinoline-6-carboxamide monohydrate, EMA/OD/160/14 Diaspirin cross-linked haemoglobin, EMA/OD/087/15 2-(2-phenylvinyl)-4-[4-methylpiperazin-1-yl]-6-(5-methyl-2H-pyrazol-3-yl-amino)-pyrimidine L(+) tartrate salt, EMA/OD/118/15 2-chloro-N6-(3-iodobenzyl)adenosine-5'-N-methyluronamide, EMA/OD/072/16 Mifamurtide, EMA/OD/052/17 N-{2-[(6-[(2,6-dichloro-3,5-

dimethoxyphenyl)carbamoyl](methyl)amino}pyrimidin-4-yl)amino]-5-(4-ethylpiperazin-1-yl)phenyl}prop-2-enamide, EMA/OD/312/16 Mertansine functionalised gold nanoconjugate, EMA/OD/038/17 Tirapazamine

Designations withdrawn: EMA/OD/013/01 Seocalcitol, EMA/OD/026/02 Doxorubicin carbon/iron magnetically targeted microparticles, EMA/OD/032/03 Nolatrexed, EMA/OD/090/07 N-[4-(3-amino-1H-indazol-4-yl)phenyl]-N'-(2-fluoro-5-methylphenyl) urea, EMA/OD/046/07 4-[3,5-bis(trimethylsilyl)benzamido] benzoic acid, EMA/OD/075/11 Brivanib alaninate, EMA/OD/031/12 Ramucirumab, EMA/OD/115/13 Tivantinib, EMA/OD/287/14 Lenvatinib

2.2.10. - EMA/OD/085/18

Treatment of hepatocellular carcinoma

Action: For discussion/adoption

Document(s) tabled:

Draft Summary report

Notes: There have been 19 designations for this condition: See 2.2.9.

2.2.11. - EMA/OD/121/18

Treatment of follicular lymphoma

Action: For discussion/adoption

Document(s) tabled:

Draft Summary report

Notes: There have been 11 designations for this condition: EMA/OD/040/06 Autologous tumor-derived immunoglobulin idiotype coupled to keyhole limpet haemocyanin, EMA/OD/065/04 Recombinant histidine-tagged idiotype immunoglobulin Fab fragment of clonal B-cell receptors, EMA/OD/158/12 lenalidomide, EMA/OD/047/13 (S)-3-(1-(9H-purin-6-ylamino)ethyl)-8-chloro-2-phenylisoquinolin-1(2H)-one, EMA/OD/111/13 Ibrutinib, EMA/OD/200/13 ¹⁷⁷Lu-tetraxetan-tetulumab, EMA/OD/013/15 obinutuzumab, EMA/OD/135/15 Autologous T cells transduced with retroviral vector encoding an anti-CD19 CD28/CD3-zeta chimeric antigen receptor, EMA/OD/103/17 Glucopyranosyl lipid A, EMA/OD/260/17 Autologous CD4+ and CD8+ T cells expressing a CD19-specific chimeric antigen receptor, EMA/OD/222/17 Tazemetostat

Designations withdrawn: EMA/OD/061/02 Iodine (131I) tositumomab, EMA/OD/079/02 Tositumomab, EMA/OD/053/13 Idelalisib

2.2.12. - EMA/OD/111/18

Treatment of peripheral T-cell lymphoma (nodal, other extranodal and leukemic/disseminated)

Action: For discussion/adoption

Document(s) tabled:

Draft Summary report

Notes: There has been 1 designation for this condition: EMA/OD/103/12 Belinostat

2.2.13. - EMA/OD/128/18

Treatment of graft-versus-host disease

Action: For discussion/adoption

Document(s) tabled:

Draft Summary report

Notes: There have been 13 designations for this condition: EMEA/OD/038/00 Inolimomab, EMEA/OD/046/05 A mixture of anti-CD3 mAb (SPV-T3a)-ricin A chain fusion protein and anti-CD7 mAb (WT1)-ricin A chain fusion protein, EMEA/OD/009/06 Methoxsalen, EMEA/OD/049/06 Budesonide (oral use), EMEA/OD/068/06 Ex-vivo cultured adult human mesenchymal stem cells, EMA/OD/022/10 Murine monoclonal antibody against CD26, EMA/OD/197/12 Allogeneic bone marrow derived mesenchymal cells expanded ex vivo in synthetic media, EMA/OD/267/14 Human plasma-derived alpha-1 proteinase inhibitor, EMA/OD/017/16 Rimiducid, EMA/OD/110/16 Cannabidiol, EMA/OD/178/16 Ibrutinib, EMA/OD/208/16 Arsenic trioxide, EMA/OD/169/17 Itacitinib

Designations withdrawn: EMEA/OD/020/00 Thalidomide, EMA/OD/118/16 Recombinant humanised monoclonal antibody against human complement component C5a

2.2.14. - EMA/OD/106/18

Treatment of cystic fibrosis

Action: For discussion/adoption

Document(s) tabled:

Draft Summary report

Notes: There have been 40 designations for this condition: See 2.2.5.

2.2.15. - EMA/OD/122/18

Treatment of transplant-associated thrombotic microangiopathy following Haematopoietic Stem Cell Transplantation

Action: For discussion/adoption

Document(s) tabled:

Draft Summary report

2.2.16. - EMA/OD/119/18

Treatment of large hemispheric infarction

Action: For discussion/adoption

Document(s) tabled:

Draft Summary report

2.2.17. - EMA/OD/108/18

Treatment of noninsulinoma pancreatogenous hypoglycaemia syndrome

Action: For discussion/adoption

Document(s) tabled:
Draft Summary report

Notes: There has been 1 designation for this condition: EMA/OD/065/16 Exendin (9-39)

2.2.18. - EMA/OD/100/18

Treatment of maple syrup urine disease

Action: For discussion/adoption

Document(s) tabled:
Draft Summary report

2.2.19. - EMA/OD/115/18

Treatment of Short Bowel Syndrome

Action: For discussion/adoption

Document(s) tabled:
Draft Summary report

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)

2.2.20. - EMA/OD/130/18

Treatment of severe combined immunodeficiency

Action: For discussion/adoption

Document(s) tabled:
Draft Summary report

2.2.21. - EMA/OD/126/18

Treatment of neuroblastoma

Action: For discussion/adoption

Document(s) tabled:
Draft Summary report

Notes: There have been 10 designations for this condition: EMEA/OD/096/07 Iodine (131I) iobenguane, EMEA/OD/093/09 16-base single-stranded PNA oligonucleotide linked to a 7-aminoacid peptide, EMA/OD/126/10 Eflornithine, EMA/OD/002/11 Chimeric monoclonal antibody against GD2, EMA/OD/020/12 16 base single stranded peptide nucleic acid oligonucleotide - 7 aminoacids peptide, EMA/OD/112/12 Chimeric monoclonal antibody against GD2, EMA/OD/199/14 Chimeric monoclonal antibody specific to O-acetyl-GD2 antigen, EMA/OD/326/14 Sodium 2-hydroxylinoleate, EMA/OD/136/15 N-[5-(3,5-difluorobenzyl)-1H-indazol-3-yl]-4-(4 methylpiperazin-1-yl)-2-(tetrahydro-2H-pyran-4-

ylamino)benzamide, EMA/OD/271/16 Iodine (131I) murine IgG1 monoclonal antibody against CD276

Designation withdrawn: EMEA/OD/013/09 Murine monoclonal antibody to GD2

2.2.22. - EMA/OD/093/18

Treatment of anti-glomerular basement membrane disease

Action: For discussion/adoption

Document(s) tabled:

Draft Summary report

2.2.23. - EMA/OD/116/18

Treatment of glioma

Action: For discussion/adoption

Document(s) tabled:

Draft Summary report

Notes: There have been 44 designations for this condition: EMA/OD/026/03 Herpes simplex virus lacking infected cell protein 34.5, EMA/OD/055/03 Gimitecan, EMA/OD/050/04 Biotinylated anti-tenascin monoclonal antibody for use with 90-Yttrium, EMA/OD/038/04 Anti epidermal growth factor receptor antibody h-R3, EMA/OD/030/05 Oligonucleotide phosphorothioate (TAAACGTTATAACGTTATGACGTCAT), sodium salt, EMA/OD/068/05 Enzastaurin hydrochloride, EMA/OD/110/05 4-[131I] iodo-L-phenylalanine, EMA/OD/081/06 Autologous dendritic cells pulsed with autologous tumour cell lysate, EMA/OD/038/07 Iodine (131I) Chlorotoxin, EMA/OD/004/08 Recombinant fusion protein of circularly-permuted IL-4 and pseudomonas exotoxin A, [IL-4(38-37)-PE38KDEL], EMA/OD/023/08 Topotecan hydrochloride (liposomal), EMA/OD/034/08 Gadodiamide (liposomal), EMA/OD/104/08 Autologous tumour-derived gp96 heat shock protein-peptide complex, EMA/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 limpet haemocyanin, EMA/OD/050/11 2-hydroxyoleic acid, EMA/OD/157/11 Adenovirus-associated vector containing human Fas-c gene, 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/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-(trifluoromethyl)benzylazanediy)bis(methylene) diquinolin-8-ol, EMA/OD/159/14 Chloroquine, EMA/OD/176/14 Adenovirus serotype 5 containing partial E1A deletion and an integrin-binding domain, EMA/OD/251/14 Recombinant human glutamate oxaloacetate transaminase 1, EMA/OD/206/15 N-(4-Methoxyphenyl)-N,2,6-trimethylfuro[2,3-d]pyrimidin-4-amine, EMA/OD/009/16 Eflornithine, EMA/OD/222/15 Delta-9-tetrahydrocannabinol and cannabidiol from extracts of the Cannabis sativa L. plant, EMA/OD/067/16 Zoledronic acid, EMA/OD/085/16 Temozolomide, EMA/OD/068/17 Picropodophyllin, EMA/OD/215/16 5-aminolevulinic acid, EMA/OD/069/17 Salmonella typhi Ty21a strain transfected with a plasmid vector encoding the human vascular endothelial growth factor receptor 2, EMA/OD/185/17 Vocimagene amiretrorepvec, EMA/OD/198/17 Flucytosine, EMA/OD/252/17 H-Arg-Pro-Lys-Pro-Gln-Gln-Phe-2Thi-Gly-Leu-Met(O₂)-NH₂-DOTA-225-actinium

Designations withdrawn: EMEA/OD/004/02 Pseudomonas exotoxin (domains II/III)- Interleukin 13 chimeric protein, EMEA/OD/074/01 Human transferrin conjugated to mutant diphtheria toxin, EMEA/OD/067/01 Carmustine (solution for intratumoral injection), EMEA/OD/050/06 Iodine (131I) anti-tenascin monoclonal antibody 81C6, EMEA/OD/037/02 Iodine (131I) anti-nucleohistone H1 chimeric biotinylated monoclonal antibody, EMEA/OD/067/03 Cilengitide, EMEA/OD/050/07 Doxorubicin hydrochloride (drug eluting beads), EMEA/OD/051/07 Irinotecan hydrochloride (drug eluting beads), 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] diazepam-11-one, EMA/OD/031/10 Glutathione-pegylated liposomal doxorubicin hydrochloride, EMA/OD/049/12 Humanised monoclonal antibody against epidermal growth factor receptor, EMA/OD/019/12 Doxorubicin (administered after synthetic double-stranded siRNA oligonucleotide directed against claudin-5 complexed with polyethyleneimine), EMA/OD/136/12 Synthetic double-stranded siRNA oligonucleotide directed against Claudin-5 complexed with polyethyleneimine (prior to administration of doxorubicin), EMA/OD/113/15 Dronabinol and cannabidiol

2.2.24. - EMA/OD/117/18

Treatment of papillary thyroid cancer

Action: For discussion/adoption

Document(s) tabled:

Draft Summary report

Notes: There have been 2 designations for this condition: EMA/OD/173/12 Lenvatinib, EMA/OD/093/13 Sorafenib tosylate

2.2.25. - EMA/OD/118/18

Treatment of anaplastic thyroid cancer

Action: For discussion/adoption

Document(s) tabled:

Draft Summary report

Notes: There has been 1 designation for this condition: EMA/OD/072/03 2-Methoxy-5-[(1Z)-2-(3,4,5-trimethoxyphenyl)ethenyl]-phenol

2.2.26. - EMA/OD/124/18

Treatment of primary mediastinal large-B-cell lymphoma

Action: For discussion/adoption

Document(s) tabled:

Draft Summary report

2.2.27. - EMA/OD/084/18

Prevention of bronchopulmonary dysplasia

Action: For discussion/adoption

Document(s) tabled:

Draft Summary report

Notes: There have been 6 designations for this condition: EMA/OD/161/13 Caffeine citrate, EMA/OD/018/14 Retinol, EMA/OD/172/13 Recombinant human surfactant protein D, EMA/OD/270/14 Recombinant human club cell 10 KDa protein, EMA/OD/010/15 Allogeneic ex-vivo-expanded human umbilical cord blood-derived mesenchymal stem cells, EMA/OD/049/17 Recombinant fragment of human surfactant protein-D

2.2.28. - EMA/OD/103/18

Treatment of Focal Segmental Glomerulosclerosis

Action: For discussion/adoption

Document(s) tabled:

Draft Summary report

Notes: There has been 1 designation for this condition: EMA/OD/115/15 4'-[(2-Butyl-4-oxo-1,3-diazaspiro[4.4]non-1-en-3-yl)methyl]-N-(4,5-dimethyl-3-isoxazolyl)-2'-(ethoxymethyl)-[1,1'-biphenyl]-2-sulfonamide

Designation withdrawn: EMA/OD/146/10 Fresolimumab

2.2.29. - EMA/OD/104/18

Treatment of haemophilia A

Action: For discussion/adoption

Document(s) tabled:

Draft Summary report

Notes: There have been 13 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/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, EMA/OD/238/16 Autologous dendritic cells incubated ex vivo with zebularine and factor VIII, EMA/OD/230/15 adeno-associated viral vector serotype 5 containing a B-domain deleted variant of human coagulation factor VIII gene, EMA/OD/093/16 Human monoclonal IgG1 antibody against tissue factor pathway inhibitor, EMA/OD/019/17 Recombinant adeno-associated viral vector serotype 6 encoding the B-domain-deleted human factor VIII, EMA/OD/189/17 Human monoclonal IgG2 antibody against tissue factor pathway inhibitor, EMA/OD/010/18 Adeno-associated viral vector serotype 8 containing a functional copy of the codon-optimised F8 cDNA encoding the B-domain deleted human coagulation factor VIII 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), EMA/OD/144/13 Humanised monoclonal modified IgG4 antibody with bispecific structure targeting factors IX, IXa, X and Xa

2.2.30. - EMA/OD/127/18

Treatment of haemophilia B

Action: For discussion/adoption

Document(s) tabled:

Draft Summary report

Notes: There have been 9 designations for this condition: 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, EMA/OD/090/11 Adeno-associated viral vector containing the human factor IX gene, 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/073/14 Recombinant factor VIIa modified with three terminal repeats derived from the β chain of human chorionic gonadotropin, EMA/OD/172/15 Adeno-associated virus viral vector serotype rh10 encoding containing the human factor IX gene, EMA/OD/018/17 Recombinant human factor IX protein modified with three point mutations, EMA/OD/116/17 Concizumab, EMA/OD/232/17 Recombinant adeno-associated viral vector containing a codon-optimized Padua derivative of human coagulation factor IX cDNA

Designations withdrawn: EMEA/OD/008/08 Pegylated recombinant factor VIIa, EMEA/OD/005/09 Pegylated recombinant human factor IX, EMEA/OD/062/09 Sequence modified human recombinant factor VIIa, EMA/OD/070/12 vatreptacog alfa (activated), EMA/OD/003/15 Adeno-associated viral vector containing the human factor IX gene

2.2.31. - EMA/OD/097/18

Treatment of alcohol-dependence

Action: For discussion/adoption

Document(s) tabled:

Draft Summary report

2.2.32. - EMA/OD/129/18

Prevention of graft versus host disease

Action: For discussion/adoption

Document(s) tabled:

Draft Summary report

Notes: There has been 1 designation for this condition: EMA/OD/248/15 Cannabidiol.

2.3. Revision of the COMP opinions

None

2.4. Amendment of existing orphan designations

None

2.5. Appeal

None

2.6. Nominations

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

Action: For adoption

Document(s) tabled:

OMP applications - appointment of rapporteurs at the 11-13 September 2018 COMP meeting

2.7. Evaluation on-going

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

Action: For information

Notes: See 7.8.1. Table 6. Evaluation Ongoing.

3. Requests for protocol assistance with significant benefit question

3.1. Ongoing procedures

3.1.1. -

Treatment of glioma

Action: For adoption

3.1.2. -

Treatment of glioma

Action: For adoption

3.1.3. -

Treatment of glioma

Action: For adoption

3.1.4. -

Prevention of graft rejection following solid organ transplantation

Action: For adoption

3.1.5. -

Treatment of transthyretin-mediated amyloidosis

Action: For adoption

3.2. Finalised letters

3.2.1. -

Treatment of bronchiolitis obliterans syndrome

Action: For information

3.2.2. -

Treatment of graft-versus-host disease

Action: For information

3.3. New requests

3.3.1. -

Treatment of naevoid basal-cell carcinoma syndrome (Gorlin syndrome)

Action: For information

3.3.2. -

Treatment of ovarian cancer

Action: For information

3.3.3. -

Treatment of spinal cord injury

Action: For information

3.3.4. -

Treatment of Leber's hereditary optic neuropathy

Action: For information

3.4. Post - Protocol assistance Issues

3.4.1. -

Treatment of Fabry disease

Action: For adoption

4. Review of orphan designation for orphan medicinal products at time of initial marketing authorisation

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

4.1.1. Symkevi - tezacaftor / ivacaftor – EMEA/H/C/004682, EMA/OD/156/16, EU/3/17/1828

Vertex Pharmaceuticals (Europe) Ltd.; Treatment of cystic fibrosis

Action: For adoption, Oral explanation to be held on Oral explanation to be held on 11 September 2018 at time 11:30

Document(s) tabled:

Draft report on review of OMPD

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

4.2.1. - voretigene neparvovec - EMEA/H/C/004451

Spark Therapeutics Ireland Ltd

a) Treatment of retinitis pigmentosa EMA/OD/040/15, EU/3/15/1518

b) Treatment of Leber's congenital amaurosis EMA/OD/150/11, EU/3/12/981

Action: For adoption, Oral explanation to be held on 12 September 2018 at time 12:00

Document(s) tabled:

Draft report on review of OMPD

4.2.2. - mexiletine hcl – EMEA/H/C/004584, EMA/OD/074/14, EU/3/14/1353

LUPIN (EUROPE) LIMITED; Treatment of myotonic disorders

Action: For discussion

Document(s) tabled:

Draft report on review of OMPD

4.2.3. - volanesorsen – EMEA/H/C/004538, EMA/OD/180/13, EU/3/14/1249

Akcea Therapeutics UK Ltd; Treatment of familial chylomicronemia syndrome

Action: For discussion

Document(s) tabled:

Draft report on review of OMPD

4.2.4. - lanadelumab – EMEA/H/C/004806, EMA/OD/075/15, EU/3/15/1551

Shire Pharmaceuticals Ireland Limited; Treatment of hereditary angioedema

Action: For discussion

Document(s) tabled:

Draft report on review of OMPD

4.2.5. - viable t-cells – EMEA/H/C/002397

Kiadis Pharma Netherlands B.V.

a) Treatment in haematopoietic stem cell transplantation EMA/OD/008/16, EU/3/16/1678

b) Treatment of acute myeloid leukaemia EMA/OD/103/14, EU/3/14/1356

c) Prevention of Graft-versus-Host Disease EMEA/OD/121/07, EU/3/08/561

Action: For discussion

Document(s) tabled:

Draft report on review of OMPD

4.2.6. - Pegylated B-domain-deleted sequence-modified recombinant human factor VIII
– EMEA/H/C/004054, EMA/OD/128/10, EU/3/10/847

Bayer AG; Treatment of haemophilia A

Action: For discussion

Document(s) tabled:

Draft report on review of OMPD

4.2.7. -mogamulizumab – EMEA/H/C/004232, EMA/OD/091/16, EU/3/16/1756

Kyowa Kirin Limited; Treatment of cutaneous T-cell lymphoma

Action: For discussion

Document(s) tabled:

Draft report on review of OMPD

4.2.8. – eteplirsen – EMEA/OD/049/08, EU/3/08/586, EMEA/H/C/004355

AVI Biopharma International Ltd; Treatment of Duchenne muscular dystrophy

Action: For information

4.3. Appeal

None

4.4. On-going procedures

Action: For information

Document(s) tabled:

Review of orphan designation for OMP for MA - On-going procedures

4.5. Orphan Maintenance Reports

Action: For information

5. Review of orphan designation for authorised orphan medicinal products at time marketing authorisation extension

5.1. After adoption of CHMP opinion

5.1.1. Kalydeco – Ivacaftor - Type II variation - EMEA/H/C/002494/II/0063/G, EMEA/OD/010/08, EU/3/08/556

Vertex Pharmaceuticals; Treatment of cystic fibrosis

CHMP rapporteur: Concepcion Prieto Yerro

Action: For adoption, Oral explanation to be held on 11 September 2018 at time 11:30

Document(s) tabled:

Draft report on review of OMPD

Sponsor's report

5.2. Prior to adoption of CHMP opinion

5.2.1. Venclyxto – Venetoclax – Type II variation – EMEA/H/C/004106/II/0008, EMA/OD/124/12, EU/3/12/1080

AbbVie Limited; Treatment of chronic lymphocytic leukaemia

CHMP rapporteur: Filip Josephson

Action: For discussion

Document(s) tabled:

Draft report on review of OMPD

Sponsor's report

5.2.2. Rubraca - rucaparib - Type II variation – EMEA/H/C/004272/II/0001, EMA/OD/085/12, EU/3/12/1049

Clovis Oncology UK Limited; Treatment of ovarian cancer

CHMP rapporteur: Jorge Camarero Jiménez

Action: For discussion

Document(s) tabled:

Draft report on review of OMPD

Sponsor's report

5.2.3. Adcetris - Brentuximab vedotin – Type II variation – EMEA/H/C/002455/II/0055, EMEA/OD/073/08, EU/3/08/596

Takeda Pharma A/S; Treatment of Hodgkin lymphoma

CHMP rapporteur: Paula Boudewina van Hennik; CHMP co-rapporteur: Jan Mueller-Berghaus;

Action: For discussion

Document(s) tabled:

Draft report on review of OMPD

Sponsor's report

5.2.4. Imnovid – pomalidomide - Type II variation – EMEA/OD/053/09, EU/3/09/672, EMEA/H/C/002682/II/0031/G

Celgene Europe Limited; Treatment of multiple myeloma

CHMP rapporteur: Robert James Hemmings

Action: For discussion

Document(s) tabled:

Draft report on review of OMPD

Sponsor's report

5.3. Appeal

None

5.4. On-going procedures

Action: For information

Document(s) tabled:

Review of orphan designation for OMP for MA extension - On-going procedures

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

None

7. Organisational, regulatory and methodological matters

7.1. Mandate and organisation of the COMP

7.1.1. Strategic Review & Learning meetings, 23-24 October 2018, Vienna, Austria

Action: For information

Documents tabled:

Draft Agenda_SRLM SAWP-COMP_Vienna 2018

7.1.2. Protocol Assistance Working Group (PAWG)

Proposed meeting time on 11 September 2018 at 13:00

Document tabled:

PAWG draft agenda for 11 September 2018 meeting

7.1.3. Non-Clinical Working Group

Proposed meeting time on 12 September 2018 at 08:30

7.1.4. Election of COMP Chairperson

Action: For adoption

Documents tabled:

[COMP Rules of Procedure EMEA/COMP/8212/00 Rev. 4](#)

Procedure for the election of the COMP chairperson

Letters of Motivation and CVs

7.1.5. Condition Working Group

Proposed meeting time on 13 September 2018 at 08:00

7.2. Coordination with EMA Scientific Committees or CMDh-v

7.2.1. Recommendations on eligibility to PRIME – report from CHMP

Action: For information

Document(s) tabled:

PRIME eligibility requests - list of adopted outcomes July 2018

7.2.2. Regulatory Science Engagement Plan to 2025

Scope: presentation of EMA's regulatory science engagement plan

Action: For discussion

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

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

PCWP/HCPWP joint meeting – 25 September 2018

Action: For information

Document(s) tabled:

Draft Agenda PCWP meeting 25 Sep 2018

Draft Agenda Joint PCWP/HCPWP meeting 25 Sep 2018

Draft Agenda HCPWP meeting 26 Sep 2018

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

Action: For information

Document(s) tabled:

Draft Agenda HCPWP meeting 26 Sep 2018

7.4. Cooperation within the EU regulatory network

7.4.1. European Commission

None

7.5. Cooperation with International Regulators

7.5.1. Food and Drug Administration (FDA)

Action: For information

Notes: Monthly teleconference

7.5.2. Japanese Pharmaceuticals and Medical Devices Agency (PMDA)

Action: For information

Notes: Ad hoc basis meeting

7.5.3. The Therapeutic Goods Administration (TGA), Australia

Action: For information

Notes: Ad hoc basis meeting

7.5.4. Health Canada

Action: For information

Notes: Ad hoc basis meeting

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

None

7.7. COMP work plan

None

7.8. Planning and reporting

7.8.1. List of all applications submitted/expected and the COMP rapporteurship distribution of valid applications submitted in 2018

Action: For information

7.8.2. Overview of orphan marketing authorisations/applications

Action: For information

8. Any other business

8.1. Telematics - Concept paper on strategy 2020-2025

Action: For discussion

8.2. EMA Business Pipeline activity and Horizon scanning

Action: For information

Document tabled:

Q3/2018 Update of the Business Pipeline report for the human scientific committees

9. Explanatory notes

The notes below give a brief explanation of the main sections and headings in the COMP agenda and should be read in conjunction with the agenda or the minutes.

Abbreviations / Acronyms

CHMP: Committee for Medicinal Product for Human Use

COMP: Committee for Orphan Medicinal Products

EC: European Commission

OD: Orphan Designation

PA: Protocol Assistance

PDCO: Paediatric Committee

PRAC: Pharmacovigilance and Risk Assessment Committee

SA: Scientific Advice

SAWP: Scientific Advice Working Party

Orphan Designation *(section 2 Applications for orphan medicinal product designation)*

The orphan designation is the appellation given to certain medicinal products under development that are intended to diagnose, prevent or treat rare conditions when they meet a pre-defined set of criteria foreseen in the legislation. Medicinal products which get the orphan status benefit from several incentives (fee reductions for regulatory procedures (including protocol assistance), national incentives for research and development, 10-year market exclusivity) aiming at stimulating the development and availability of treatments for patients suffering from rare diseases.

Orphan Designations are granted by Decisions of the European Commission based on opinions from the COMP. Orphan designated medicinal products are entered in the Community Register of Orphan Medicinal Products.

Protocol Assistance *(section 3 Requests for protocol assistance with significant benefit question)*

The protocol assistance is the help provided by the Agency to the sponsor of an orphan medicinal product, on the conduct of the various tests and trials necessary to demonstrate the quality, safety and efficacy of the medicinal product in view of the submission of an application for marketing authorisation.

Sponsor

Any legal or physical person, established in the Community, seeking to obtain or having obtained the designation of a medicinal product as an orphan medicinal product.

Maintenance of Orphan Designation *(section 4 Review of orphan designation for orphan medicinal products for marketing authorisation).*

At the time of marketing authorisation, the COMP will check if all criteria for orphan designation are still met. The designated orphan medicinal product should be removed from the Community Register of Orphan Medicinal Products if it is established that the criteria laid down in the legislation are no longer met.

More detailed information on the above terms can be found on the EMA website: www.ema.europa.eu/