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SCIENCE MEDICINES HEALTH

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Inspections, Human Medicines Pharmacovigilance and Committees

Committee for Orphan Medicinal Products (COMP)

Draft agenda for the meeting on 5-7 December 2017

Chair: Bruno Sepodes – Vice-Chair: Lesley Greene

5 December 2017, 08:30-19:30, room 2F

6 December 2017, 08:30-19:30, room 2F

7 December 2017, 08:30-18:30, room 2F

Health and safety information

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

Disclaimers

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

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

Note on access to documents

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



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1. Introduction

1.1. Welcome and declarations of interest of members and experts

Pre-meeting list of participants and restrictions in relation to declarations of interests applicable to the items of the agenda for the COMP plenary session to be held 05-07 December 2017. See December 2017 COMP minutes (to be published post 16-18 January 2018 COMP meeting).

1.2. Adoption of agenda

COMP agenda for 05-07 December 2017.

1.3. Adoption of the minutes

COMP minutes for 30-31 October 2017.

2. Applications for orphan medicinal product designation

2.1. For opinion

2.1.1. - EMA/OD/166/17

Treatment of small cell lung cancer

Action: For adoption, Oral explanation to be held on 6 December 2017 at 09:30

Document(s) tabled:

Draft Summary report with response to LoQs

Notes: There have been 6 designations for this condition: EMA/OD/086/14 2-(2-methyl-5-nitro-1H-imidazol-1-yl)ethylsulfamide, EMA/OD/015/16 Rovalpituzumab tesirine, EMA/OD/056/10 Maytansinoid-conjugated humanised monoclonal antibody against CD56, EMEA/OD/113/07 Amrubicin hydrochloride, EMEA/OD/055/07 Picoplatin, EMEA/OD/056/04 Sabarubicin

2.1.2. - EMA/OD/164/17

Treatment of congenital adrenal hyperplasia

Action: For adoption, Oral explanation to be held on 6 December 2017 at 11:00

Document(s) tabled:

Draft Summary report with response to LoQs

Notes: There has been 1 designation for this condition: EMEA/OD/020/05 Hydrocortisone (modified release tablet)

Designation withdrawn: EMA/OD/063/15 Verucerfont

2.1.3. - EMA/OD/160/17

Treatment of cerebral autosomal-dominant arteriopathy with subcortical infarcts and leukoencephalopathy

Action: For adoption, Oral explanation to be held on 6 December 2017 at 12:00

Document(s) tabled:

Draft Summary report with response to LoQs

2.1.4. - EMA/OD/144/17

Treatment of oculopharyngeal muscular dystrophy (OPMD)

Action: For adoption, Oral explanation to be held on 6 December 2017 at 14:30

Document(s) tabled:

Draft Summary report with response to LoQs

2.1.5. - EMA/OD/161/17

Treatment of haematopoietic stem cell transplantation

Action: For adoption, Oral explanation to be held on 5 December 2017 at 12:00

Document(s) tabled:

Draft Summary report with response to LoQs

Notes: There have been 6 designations for this condition: EMA/OD/090/16 Radio-iodinated (131I) anti-CD45 murine monoclonal antibody, EMA/OD/008/16 Donor T lymphocytes depleted ex vivo of host alloreactive T cells using photodynamic treatment, EMA/OD/020/16 Allogeneic donor-derived ex-vivo expanded T lymphocytes transduced with a retroviral vector containing inducible caspase 9 and truncated CD19, EMA/OD/191/16 Human donor haematopoietic stem and progenitor cells that have been treated ex vivo with the protein transduction domain of the HIV-1 transactivation protein fused to MYC transcription factor, EMA/OD/149/16 Allogeneic peripheral blood mononuclear cells incubated ex vivo with 16, 16-dimethyl prostaglandin E2 and dexamethasone, EMA/OD/257/16 Allogeneic, ex vivo expanded, umbilical cord blood-derived, hematopoietic CD34+ progenitor cells and allogeneic, non-expanded, umbilical cord blood-derived, hematopoietic mature myeloid and lymphoid cells

2.1.6. - EMA/OD/159/17

Treatment of haematopoietic stem cell transplantation

Action: For adoption, Oral explanation to be held on 5 December 2017 at 12:00

Document(s) tabled:

Draft Summary report with response to LoQs

Notes: There have been 6 designations for this condition: Please see 2.1.5.

2.1.7. - EMA/OD/167/17

Treatment of mucopolysaccharidosis type I

Action: For adoption, Oral explanation to be held on 6 December 2017 at 17:00

Document(s) tabled:

Draft Summary report with response to LoQs

Notes: There have been 4 designations for this condition: EMA/OD/121/14 Pentosan polysulfate sodium, EMA/OD/165/14 Ataluren, EMA/OD/138/14 Recombinant human insulin receptor monoclonal antibody-fused- α -L-iduronidase, EMA/OD/119/16 6'-(R)-methyl-5-O-(5-amino-5,6-dideoxy- α -L-talofuranosyl)-paromamine sulfate

2.1.8. - EMA/OD/168/17

Treatment of mucopolysaccharidosis type II

Action: For adoption, Oral explanation to be held on 6 December 2017 at 17:00

Document(s) tabled:

Draft Summary report with response to LoQs

Notes: There have been 3 designations for this condition: EMA/OD/076/15 Adeno-associated viral vector serotype 9 containing the human iduronate-2-sulfatase gene, EMA/OD/091/13 Recombinant human insulin receptor monoclonal antibody-fused iduronate 2-sulfatase, EMA/OD/056/01 Iduronate-2-sulfatase

2.1.9. - EMA/OD/096/17

Treatment of adult-onset Still's disease

Action: For information

Document(s) tabled:

Withdrawal request of 17 November 2017

2.1.10. - EMA/OD/129/17

Treatment of systemic juvenile idiopathic arthritis

Action: For information

Document(s) tabled:

Withdrawal request of 16 November 2017

2.1.11. - EMA/OD/155/17

Treatment of Immunoglobulin G4-Related Disease

Action: For adoption, Oral explanation to be held on 7 December 2017 at 09:30

Document(s) tabled:

Draft Summary report with response to LoQs

2.1.12. - EMA/OD/152/17

Treatment of atypical haemolytic uremic syndrome

Action: For information

Document(s) tabled:

Withdrawal request of 14 November 2017

Notes:

There has been 1 designation for this condition: EMA/OD/027/09 Eculizumab

2.1.13. - EMA/OD/151/17

Treatment of Huntington's disease

Action: For adoption, Oral explanation to be held on 7 December 2017 at 12:00

Document(s) tabled:

Draft Summary report with response to LoQs

Notes: There have been 6 designations for this condition: EMA/OD/021/00 Ethyl Eicosapentaenoate, EMA/OD/011/05 4-[3-(methylsulfonyl)phenyl]-1-propylpiperidine x HCl, EMA/OD/066/09 6-chloro-2,3,4,9-tetrahydro-1H-carbazole-1-carboxamide, EMA/OD/095/09 Lithium citrate tetrahydrate (in reverse- micelle formulation), EMA/OD/192/14 2'-O-methyl phosphorothioate RNA oligonucleotide, 5' m5CUGm5CUGm5CUGm5CUGm5CUGm5CUGm5CUG-3', EMA/OD/066/16 2-[4-(1-methyl-4-pyridin-4-yl-1H-pyrazol-3-yl)-phenoxyethyl]-quinoline succinic acid
Designation withdrawn: EMA/OD/061/08 2,3,4,5 tetrahydro-2,8-dimethyl-5-[2-(6-methyl-3-pyridinyl)ethyl]-1H-pyrido[4,3-b]indole dihydrochloride

2.1.14. - EMA/OD/095/17

Treatment of pancreatic cancer

Action: For information

Document(s) tabled:

Withdrawal request of 17 November 2017

Notes:

There have been 36 designations for this condition: EMA/OD/055/02 G17(9) gastrin-Diphtheria Toxoid Conjugate, EMA/OD/068/02 Rubitecan, EMA/OD/009/05 Bovine bile extract, EMA/OD/028/05 4-imino-1, 3-diazobicyclo-[3.1.0]-hexan-2-one, EMA/OD/063/06 Paclitaxel (liposomal), EMA/OD/026/06 Human telomerase reverse transcriptase peptide (611-626), EMA/OD/103/06 Cisplatin (liposomal), EMA/OD/100/08 L-asparaginase encapsulated in erythrocytes, EMA/OD/006/08 Nimotuzumab, EMA/OD/080/08 Yttrium (90Y)-DOTA-radiolabelled humanized monoclonal antibody against mucin 1, EMA/OD/101/08 S-[2,3-bispalmitoyloxy-(2R)-propyl]-cysteiny-GNNDENISFKEK, EMA/OD/030/09 Trabedersen, EMA/OD/105/09 Brivudine, EMA/OD/069/09 N-[(2S)-2,3-dihydroxypropyl]-3-[(2-fluoro-4-iodophenyl)amino]isonicotinamide hydrochloride, EMA/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/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/071/13 Chimeric monoclonal antibody against claudin-18 splice variant 2, EMA/OD/164/13 Cysteamine bitartrate, EMA/OD/081/14 Immunoglobulin G1 (TEXT TOO LONG), EMA/OD/085/14 [5-Amino-1-(4-fluoro-phenyl)-1H-pyrazol-4-yl]-[3-(2,3-dihydroxy-propoxy)-phenyl]-methanone, EMA/OD/187/14 Herpes simplex type 1 virus containing cellular B-myb gene as tumour-specific promoter, EMA/OD/143/14 Heat-killed Mycobacterium obuense (whole cell), EMA/OD/173/14 Pegylated recombinant human hyaluronidase PH20, EMA/OD/302/14 Human reovirus type 3 Dearing strain, EMA/OD/034/15 Modified adenovirus serotype 5/35 containing a CMV promoter-driven transgene cassette with the human transgenes for a membrane-bound CD40 ligand (TMZ-CD40L) and full length 4-1BBL, EMA/OD/168/15 Live attenuated Listeria monocytogenes delta actA/delta 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 (TEXT TOO LONG)

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/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. For discussion / preparation for an opinion

2.2.1. - EMA/OD/195/17

Prevention of radiotherapy-induced oral mucositis in head and neck cancer patients

Action: For discussion/adoption

Document(s) tabled:

Draft Summary report

2.2.2. - EMA/OD/188/17

Treatment of chronic myeloid leukaemia

Action: For discussion/adoption

Document(s) tabled:

Draft Summary report

Notes:

There have been 7 designations for this condition: Bafetinib EMEA/OD/143/09, Allogeneic ex vivo expanded umbilical cord blood cells EMEA/OD/035/09, 17-allylamino-17-demethoxygeldanamycin EMEA/OD/079/04

Val-Leu-Gln-Glu-Leu-Asn-Val-Thr-Val (Pr1 nanopeptide, sequence 169-177, of proteinase 3) EMEA/OD/060/04, Homoharringtonine EMEA/OD/041/04, Nilotinib EMEA/OD/003/06, Benzamide, 3-(2-imidazo[1,2-b]pyridazin-3-ylethynyl)-4-methyl-N-[4-[(4-methyl-1-piperazinyl)methyl]-3-(trifluoromethyl)phenyl]- EMEA/OD/121/09.

2.2.3. - EMA/OD/193/17

Treatment of acute myeloid leukaemia

Action: For discussion/adoption

Document(s) tabled:

Draft Summary report

Notes: There have been 53 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-methyl 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/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/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 pyrrolobenzodiazepine dimer drug, EMA/OD/112/15 Recombinant human interleukin-3 truncated diphtheria toxin fusion protein, EMA/OD/145/15 Humanised monoclonal antibody of the IgG4 kappa isotype targeting CD47, EMA/OD/165/15 Sodium (2R,3S,5R)-5-(4-amino-2-oxo-1,3,5-triazin-1(2H)-yl)-2-(hydroxymethyl)tetrahydrofuran-3-yl ((2R,3S,5R)-5-(2-amino-6-oxo-1H-purin-9(6H)-yl)-3-hydroxytetrahydrofuran-2-yl)methyl phosphate, EMA/OD/144/15 Combretastatin A1-diphosphate, 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-Ph, 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 Designations withdrawn: EMEA/OD/065/02 2-chloro-9-[2-deoxy-2-fluoro-β-D-arabinofuranosyl]adenine, EMEA/OD/051/04 Homoharringtonine, 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/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

2.2.4. - EMAOD/170/17

Treatment of soft tissue sarcoma

Action: For discussion/adoption

Document(s) tabled:

Draft Summary report

Notes: There have been 16 designations for this condition: EMEA/OD/001/01 Ecteinascidin 743, EMEA/OD/042/06 Doxorubicin hydrochloride (liposomal), EMA/OD/129/16 Crenolanib besylate, EMA/OD/155/11 Yttrium (90Y)-DTPA-radiolabelled chimeric monoclonal antibody against frizzled homologue 10, 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/037/16 16-base single-stranded

peptide nucleic acid oligonucleotide linked to a 7 aminoacid peptide, EMA/OD/266/14 Olaratumab, EMA/OD/159/15 Glucopyranosyl lipid A stable emulsion and recombinant New York esophageal squamous cell carcinoma-1 protein, EMA/OD/184/15 (S)-N-(5-((R)-2-(2,5-difluorophenyl)pyrrolidin-1-yl)pyrazolo[1,5-a]pyrimidin-3-yl)-3-hydroxypyrrolidine-1-carboxamide hydrogen sulfate, EMA/OD/215/15 Human/murine chimeric monoclonal antibody against endoglin, EMA/OD/238/15 Sindbis virus envelope pseudotyped lentiviral vector encoding New York esophageal squamous cell carcinoma-1 protein, EMA/OD/064/16 Autologous CD4+ and CD8+ T cells transduced with lentiviral vector containing an affinity-enhanced T-cell receptor targeting the New York esophageal antigen-1, EMA/OD/108/16 A non-covalent trimer of tumor necrosis factor fused to an antibody specific to the extra-domain B of fibronectin in single-chain variable fragment format, EMA/OD/166/16 Propranolol, EMA/OD/201/16 (3'R,4'S,5'R)-N-[(3R,6S)-6-carbamoyltetrahydro-2H-pyran-3-yl]-6"-chloro-4'-(2-chloro-3-fluoropyridin-4-yl)-4,4-dimethyl-2"-oxo-1",2"-dihydrodispiro[cyclohexane-1,2'-pyrrolidine-3',3"-indole]-5'-carboxamide mono(4-methylbenzenesulfonate)

Designations withdrawn: EMEA/OD/059/03 N-acetylsarcosyl-glycyl-L-valyl-D-alloisoleucyl-L-threonyl-L-norvalyl-L-isoleucyl-L-arginyl-L-prolyl-N-ethylamide, EMEA/OD/050/05 (1R, 2R, 4S)-4-{(2R)-2-[(3S,6R,7E,9R,10R,12R,14S,15E,17E,19E,21S,23S,26R, 27R,34aS)-9,27-dihydroxy-10,21-dimethoxy-6,8,12,14,20,26-hexamethyl-1,5,11,28,29-pentaoxo-1,4,5,6,9,10,11,12,13,14,21,22,23,24,25,26,27,28,29,31,32,33,34,34a-tetra-cosahydro-3H-23,27-epoxyprido[2,1-c][1,4]oxazacyclohentracontin-3-yl]propyl}-2-methoxy-cyclohexyldimethyl-phosphinate, EMEA/OD/071/05 Brostallicin, EMEA/OD/083/06 Fenretinide, EMEA/OD/044/08 Palifosfamide, EMA/OD/141/10 Ombrabulin, EMA/OD/110/11 (1-methyl-2-nitro-1H-imidazole-5-yl)methyl N,N'-bis(2-bromoethyl) diamidophosphate

2.2.5. - EMA/OD/192/17

Treatment in haematopoietic stem cell transplantation

Action: For discussion/adoption

Document(s) tabled:

Draft Summary report

Notes: There have been 6 designations for this condition: EMA/OD/008/16 Donor T lymphocytes depleted ex vivo of host alloreactive T cells using photodynamic treatment, EMA/OD/020/16 Allogeneic donor-derived ex-vivo expanded T lymphocytes transduced with a retroviral vector containing inducible caspase 9 and truncated CD19, EMA/OD/090/16 Radio-iodinated (131I) anti-CD45 murine monoclonal antibody, EMA/OD/149/16 Allogeneic peripheral blood mononuclear cells incubated ex vivo with 16, 16-dimethyl prostaglandin E2 and dexamethasone, EMA/OD/191/16 Human donor haematopoietic stem and progenitor cells that have been treated ex vivo with the protein transduction domain of the HIV-1 transactivation protein fused to MYC transcription factor, EMA/OD/257/16 Allogeneic, ex vivo expanded, umbilical cord blood-derived, hematopoietic CD34+ progenitor cells and allogeneic, non-expanded, umbilical cord blood-derived, hematopoietic mature myeloid and lymphoid cells

2.2.6. - EMA/OD/173/17

Treatment of cystic fibrosis

Action: For discussion/adoption

Document(s) tabled:

Draft Summary report

Notes: There have been 39 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/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

2.2.7. - EMA/OD/305/16

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/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-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
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 Epoprostenol, 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/014/10 Pyr-His-Trp-Ser-Tyr-D-Lys(doxorubicinylglutarate)-Leu-Arg-Pro-Gly-NH₂, acetate salt, EMA/OD/094/11 Vincalutamide, EMA/OD/094/11 Vincalutamide-23-oic acid, O4-deacetyl-2-[(2-mercaptoethoxy)carbonyl]hydrazide, disulfide with N-[4-[(2-amino-3,4-dihydro-4-oxo-6-pteridinyloxy)methyl]amino]benzoyl]-L-gamma-glutamyl-L-alpha-aspartyl-L-arginyl-L-alpha-aspartyl-L-alpha-aspartyl-L-cysteine, 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

2.2.8. - EMA/OD/165/17

Treatment of tuberous sclerosis

Action: For discussion/adoption

Document(s) tabled:

Draft Summary report

Notes: There have been 4 designations for this condition: EMA/OD/010/10 Everolimus, EMA/OD/100/15 Sirolimus, EMA/OD/007/17 Sirolimus, EMA/OD/047/17 Sirolimus

2.2.9. - EMA/OD/150/17

Treatment of graft-versus-host disease

Action: For discussion/adoption

Document(s) tabled:

Draft Summary report

Notes: There have been 12 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

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

2.2.10. - EMA/OD/186/17

Treatment of congenital erythropoietic porphyria

Action: For discussion/adoption

Document(s) tabled:

Draft Summary report

Notes: There has been 1 designation for this condition: EMEA/OD/019/08 [N1e4, D-Phe7]-alfa-melanocyte stimulating hormone

2.2.11. - EMA/OD/178/17

Treatment of Stargardt's disease

Action: For discussion/adoption

Document(s) tabled:
Draft Summary report

Notes: There have been 6 designations for this condition: EMEA/OD/127/09 Lentiviral vector containing the human ABCA4 gene, EMA/OD/173/10 Human embryonic stem-cell-derived retinal pigment epithelial cells, EMA/OD/175/12 Ramiprilat, EMA/OD/124/13 Soraprazan, EMA/OD/005/14 Mixture of two adeno-associated viral vectors of serotype 8 containing the 5'-half sequence of human ABCA4 gene and the 3'-half sequence of human ABCA4 gene, EMA/OD/295/14 Ecothiopate iodide
Designation withdrawn: EMEA/OD/084/08 Adeno-associated viral vector serotype 5 containing the human ABCA4 gene

2.2.12. - EMA/OD/198/17

Treatment of glioma

Action: For discussion/adoption

Document(s) tabled:
Draft Summary report

Notes: There have been 41 designations for this condition: EMEA/OD/026/03 Herpes simplex virus lacking infected cell protein 34.5, EMEA/OD/055/03 Gimitecan, EMEA/OD/050/04 Biotinylated anti-tenascin monoclonal antibody for use with 90-Yttrium, EMEA/OD/038/04 Anti epidermal growth factor receptor antibody h-R3, EMEA/OD/030/05 Oligonucleotide phosphorothioate (TAAACGTTATAACGTTATGACGTCAT), sodium salt, EMEA/OD/068/05 Enzastaurin hydrochloride, EMEA/OD/110/05 4-[131I] iodo-L-phenylalanine, EMEA/OD/081/06 Autologous dendritic cells pulsed with autologous tumour cell lysate, EMEA/OD/038/07 Iodine (131I) Chlorotoxin, EMEA/OD/004/08 Recombinant fusion protein of circularly-permuted IL-4 and pseudomonas exotoxin A, [IL-4(38-37)-PE38KDEL], EMEA/OD/023/08 Topotecan hydrochloride (liposomal), EMEA/OD/034/08 Gadodiamide (liposomal), EMEA/OD/104/08 Autologous tumour-derived gp96 heat shock protein-peptide complex, EMEA/OD/098/09 Recombinant fusion protein consisting of the extracellular portion of CD95 fused to the Fc part of a human IgG1 molecule, EMA/OD/086/10 7-beta-hydroxycholesteryl-3-beta-oleate, EMA/OD/092/12 IL-12-secreting dendritic cells, loaded with autologous tumour lysate, EMA/OD/077/11 L-cysteine, L-leucyl-L-alpha-glutamyl-L-alpha-glutamyl-L-lysyl-L-lysylglycyl-L-asparaginyll-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 lim, 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 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

2.2.13. - EMA/OD/175/17

Treatment of acute myeloid leukaemia

Action: For discussion/adoption

Document(s) tabled:

Draft Summary report

Notes: There have been 53 designations for this condition: Please see 2.2.3.

2.2.14. - EMA/OD/171/17

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)

2.2.15. - EMA/OD/189/17

Treatment of haemophilia A

Action: For discussion/adoption

Document(s) tabled:

Draft Summary report

Notes: There have been 11 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

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

2.2.16. - EMA/OD/190/17

Treatment of haemophilia B

Action: For discussion/adoption

Document(s) tabled:

Draft Summary report

Notes: There have been 7 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

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

2.2.17. - EMA/OD/177/17

Treatment of LIPIN1 disease

Action: For discussion/adoption

Document(s) tabled:

Draft Summary report

2.2.18. - EMA/OD/169/17

Treatment of graft-versus-host disease

Action: For discussion/adoption

Document(s) tabled:

Draft Summary report

Notes: There have been 12 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

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

2.2.19. - EMA/OD/191/17

Treatment in cardiopulmonary by-pass

Action: For discussion/adoption

Document(s) tabled:

Draft Summary report

2.2.20. - EMA/OD/312/16

Treatment of hepatocellular carcinoma

Action: For discussion/adoption

Document(s) tabled:

Draft Summary report

Notes: There have been 21 designations for this condition: EMEA/OD/015/02 Thymalfasin, EMEA/OD/087/04 Pegylated arginine deiminase, EMEA/OD/048/04 Doxorubicine polyisohexylcyanoacrylate nanoparticles, EMEA/OD/018/05 Nemorubicin hydrochloride, EMEA/OD/109/05 Sorafenib tosylate, EMEA/OD/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/115/13 Tivantinib, EMA/OD/160/14 Diaspirin cross-linked haemoglobin, EMA/OD/287/14 Lenvatinib, 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/038/17 Tirapazamine

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

2.2.21. - EMA/OD/154/17

Treatment of Duchenne muscular dystrophy

Action: For discussion/adoption

Document(s) tabled:

Draft Summary report

Notes: There have been 26 designations for this condition: 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-dimethylam, 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-dimethylaminophosphonamidate], 3'-[2'a-[N2-acetyl-L-arginyl-6-aminohexanoyl-L-arginyl-L-arginyl-β-alanyl-L-arginyl-L-arginyl-6-aminohexanoyl-L-arginyl-L-arginyl-β-alanyl-L-arginyl-6-aminohexanoyl-β-alanyl], octahydrochloride, EMA/OD/090/13 Naproxcinod, EMA/OD/142/11 Exon 45 specific phosphorothioate oligonucleotide, EMA/OD/143/11 Exon 53 specific phosphorothioate oligonucleotide, 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 17 α ,21-dihydroxy-16 α -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, EMA/OD/109/15 N-(2-((4Z,7Z,10Z,13Z,16Z,19Z)-docosa-4,7,10,13,16,19-hexaenamido)ethyl)-2-hydroxybenzamide, EMA/OD/161/16 Recombinant adeno-associated viral vector encoding a human micro-dystrophin gene under the control of a muscle specific promoter, EMA/OD/096/16 Adeno-associated viral vector serotype 9 containing the human mini-dystrophin gene

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

2.2.22. - EMA/OD/179/17

Treatment of congenital adrenal hyperplasia

Action: For discussion/adoption

Document(s) tabled:

Draft Summary report

Notes: There has been 1 designation for this condition: EMEA/OD/020/05 Hydrocortisone (modified release tablet)

Designation withdrawn: EMA/OD/063/15 Verucerfont

2.2.23. - EMA/OD/180/17

Treatment of acromegaly

Action: For discussion/adoption

Document(s) tabled:

Draft Summary report

Notes: There have been 5 designations for this condition: EMEA/OD/010/09 Octreotide chloride (lipid depot solution), EMEA/OD/051/09 Pasireotide, EMA/OD/107/12 Cyclo(-gamma-aminobutyryl-L-phenylalanyl-L-tryptophanyl-D-tryptophanyl-L-lysyl-L-threonyl-L-phenylalanyl-N-3-carboxypropyl)-glycine amide, acetate salt, EMA/OD/042/13 Octreotide acetate (oral use), EMA/OD/023/16 2'-O-(2-methoxyethyl) phosphorothioate antisense oligonucleotide targeting the growth hormone receptor

Designation withdrawn: EMA/OD/108/11 Recombinant protein consisting of modified human growth hormone releasing hormone and the translocation and endopeptidase domains of botulinum toxin serotype D

2.2.24. - EMA/OD/182/17

Treatment of GM2 gangliosidosis

Action: For discussion/adoption

Document(s) tabled:

Draft Summary report

Notes: There has been 1 designation for this condition: Acetylleucine EMA/OD/158/17

2.2.25. - EMA/OD/176/17

Treatment of Myasthenia Gravis

Action: For discussion/adoption

Document(s) tabled:

Draft Summary report

Notes: There have been 3 designations for this condition: EMEA/OD/078/09 Peptides mimicking antigen receptors on autoimmune B cells and autoimmune T cells associated with myasthenia gravis, EMA/OD/062/14 Eculizumab, EMA/OD/318/14 Fusion proteins composed by a genetically modified Cholera Toxin Subunit A1, peptides from the acetylcholine receptor alpha chain and a dimer of the D fragment from Staphylococcus aureus protein A
Designations withdrawn: EMEA/OD/008/04 5'-CTG CCA CGT TCT CCT GC-(2' methoxy)A-(2' methoxy)C-(2' methoxy)C-3', EMEA/OD/036/06 H-Val-Ile-Val-Lys-Leu-Ile-Pro-Ser-Thr-Ser-Ser-Ala-Val-Asp-Thr-Pro-Tyr-Leu-Asp-Ile-Thr-Tyr-His-Phe-Val-Ala-Gln-Arg-Leu-Pro-Leu-OH

2.2.26. - EMA/OD/184/17

Treatment of sickle cell disease

Action: For discussion/adoption

Document(s) tabled:

Draft Summary report

Notes: There have been 12 designations for this condition: EMEA/OD/017/05 Extract of Sorghum bicolor leaf, Pterocarpus osun stem, Piper guineense seed and Caryophylli flower, EMEA/OD/107/08 2,2-dimethylbutyric acid, sodium salt, EMEA/OD/075/09 Pegylated carboxyhaemoglobin, EMA/OD/016/12 Levoglutamide, EMA/OD/040/12 Human Erythrocytes encapsulating Inositol Hexaphosphate, EMA/OD/026/12 Humanised monoclonal antibody targeting P-selectin, EMA/OD/084/13 (1R,3R,4R,5S)-3-O-[2-O-benzoyl-3-O-(sodium(2S)-3-cyclohexyl-propanoate-2-yl)-β-D-galactopyranosyl]-4-O-(α-L-fucopyranosyl)-5-orothylamido-cyclohexane-1-carboxylic acid (ethyl-2-amidyl-ethyloxy-2-acetyl-(8-amino-1,3,6-naphthalene-tris sodium sulfonate) amide, EMA/OD/184/13 Autologous CD34+ haematopoietic stem cells transduced with lentiviral vector encoding the human beta A-T87Q-globin gene, EMA/OD/210/14 Sevufparin sodium, EMA/OD/187/16 2-hydroxy-6-((2-(1-isopropyl-1H-pyrazol-5-yl)pyridin-3-yl) methoxy)benzaldehyde, EMA/OD/144/16 Synthetic human hepcidin, EMA/OD/008/17 Decitabine and tetrahydrouridine
Designations withdrawn: EMA/OD/162/12 Poloxamer 188, EMA/OD/249/14 5-hydroxymethyl-2-furfural

2.2.27. - EMA/OD/145/17

Treatment of Pontocerebellar Hypoplasia Type 6

Action: For discussion/adoption

Document(s) tabled:

Draft Summary report

2.2.28. - EMA/OD/185/17

Treatment of glioma

Action: For discussion/adoption

Document(s) tabled:

Draft Summary report

Notes: There have been 41 designations for this condition: Please see 2.2.12.

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 coordinators

Action: For adoption

Document(s) tabled:

OMP applications - appointment of coord. at the 05-07 December 2017 COMP meeting

2.7. Evaluation on-going

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

Action: For information

Notes: See 7.8.1. Evaluation Ongoing.

3. Requests for protocol assistance with significant benefit question

3.1. Ongoing procedures

3.1.1. -

Treatment of spinal muscular atrophy

Action: For adoption

3.1.2. -

Treatment of plasma cell myeloma

Action: For adoption

3.1.3. -

Treatment of sickle cell disease

Action: For adoption

3.1.4. -

Treatment of ornithine transcarbamylase deficiency

Action: For adoption

3.1.5. -

Treatment of Lennox-Gastaut syndrome

Action: For adoption

3.1.6. -

Treatment of mantle cell lymphoma

Action: For adoption

3.1.7. -

Treatment of acute myeloid leukaemia

Action: For adoption

3.1.8. -

Treatment of myelodysplastic syndromes Karri Penttilä;

Action: For adoption

3.1.9. -

Treatment of Leber's hereditary optic neuropathy

Action: For adoption

3.2. Finalised letters

3.2.1. -

Treatment of chronic lymphocytic leukaemia

Action: For information

3.2.2. -

Treatment of idiopathic pulmonary fibrosis

Action: For information

3.2.3. -

Treatment of small cell lung cancer

Action: For information

3.3. New requests

3.3.1. -

Treatment of Niemann-Pick disease, type C

Action: For information

3.3.2. -

Treatment of mucopolysaccharidosis type I

Action: For information

3.3.3. -

TKI inhibitor for treatment of gastrointestinal stromal tumors

Action: For information

3.3.4. -

Treatment of cutaneous T-cell lymphoma

Action: For information

3.3.5. -

Treatment of diffuse large B-cell lymphoma

Action: For information

3.3.6. -

Treatment of multiple myeloma

Action: For information

3.3.7. -

Treatment of glioma

Action: For information

3.3.8. -

Treatment of multiple myeloma

Action: For information

3.3.9. -

Treatment of paroxysmal nocturnal haemoglobinuria

Action: For information

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. Jorveza - budesonide - EMEA/H/C/004655, EMA/OD/078/13, EU/3/13/1181

Dr. Falk Pharma GmbH; Treatment of eosinophilic esophagitis

Action: For adoption, Oral explanation to be held on 5 December 2017 at time 11:00

Document(s) tabled:

Draft report on review of OMPD

Notes: Accelerated CHMP assessment

Status of the procedure at the CHMP: CHMP opinion adopted in November 2017

4.1.2. [Adcetris - Brentuximab vedotin – Type II variation - EMA/OD/100/11, EU/3/11/939, EMEA/H/C/002455/II/0048](#)

Takeda Pharma A/S - Denmark; Treatment of cutaneous T-cell lymphoma

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

Action: For adoption, Oral explanation to be held on 5 December 2017 at time 15:30

Document(s) tabled:

Draft report on review of OMPD

Notes: Status of the procedure at the CHMP: CHMP opinion adopted in November 2017

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

4.2.1. [Lenvima - Lenvatinib – Type II variation - EMEA/H/C/003727/II/0011/G, EMA/OD/287/14, EU/3/15/1460](#)

Eisai Ltd; Treatment of hepatocellular carcinoma

CHMP rapporteur: Bart Van der Schueren; CHMP co-rapporteur: Robert James Hemmings

Action: For information

Document(s) tabled:

Draft report on review of OMPD

4.2.2. [- plitidepsin – EMEA/H/C/004354, EMEA/OD/044/04, EU/3/04/245](#)

Pharma Mar SA; Treatment of multiple myeloma

Action: For information

4.2.3. [- expanded human allogeneic mesenchymal adult stem cells extracted from adipose tissue – EMEA/H/C/004258, EMEA/OD/054/09, EU/3/09/667](#)

TIGENIX, S.A.U.; Treatment of anal fistula

Action: For adoption, Oral explanation to be held on 5 December 2017 at time 14:30

Document(s) tabled:

Draft report on review of OMPD

CHMP assessment report

4.2.4. [- rucaparib - EMEA/H/C/004272, EMA/OD/085/12, EU/3/12/1049](#)

Clovis Oncology UK Ltd; Treatment of ovarian cancer

Action: For information

Document(s) tabled:

Draft report on review of OMPD

4.2.5. - velmanase alfa - EMEA/H/C/003922, EMEA/OD/074/04, EU/3/04/260,

Chiesi Farmaceutici S.p.A.; Treatment of alpha-Mannosidosis

Action: For information

Document(s) tabled:

Draft report on review of OMPD

4.2.6. - glibenclamide - EMEA/H/C/004379, EMA/OD/149/15, EU/3/15/1589

Ammtek; Treatment of neonatal diabetes

Action: For discussion

Document(s) tabled:

Draft report on review of OMPD

4.2.7. - burosumab - EMEA/H/C/004275, EMEA/H/C/004275, EU/3/14/1351

Kyowa Kirin Limited; Treatment of X-linked hypophosphataemia

Action: For discussion

Document(s) tabled:

Draft report on review of OMPD

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. Public Summary of Opinions

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. Nplate - Recombinant megakaryopoiesis-stimulating protein Romiplostim –Type II variation - EMEA/OD/008/05, EU/3/05/283, EMEA/H/C/000942/II/0060/G

Amgen Europe BV; Treatment of idiopathic thrombocytopenic purpura

CHMP rapporteur: Concepcion Prieto Yerro; CHMP co-rapporteur: Paula Boudewina van Hennik

Action: For information

Document(s) tabled:

Draft report on review of OMPD

Notes:

Status of the procedure at the CHMP: CHMP positive opinion adopted in November 2017

5.2. Prior to adoption of CHMP opinion

5.2.1. Bosulif (Bosutinib) - Type II variation – EMEA/H/C/002373/II/0025/G, EMEA/OD/160/09, EU/3/10/762

Pfizer Limited - UK; Treatment of chronic myeloid leukaemia

CHMP rapporteur: Harald Enzmann

Action: For information

Document(s) tabled:

Draft report on review of OMPD

Sponsor's report

5.2.2. Lynparza - (Olaparib) – EMEA/H/C/003726/X/0016/G, EMEA/OD/063/07, EU/3/07/501

AstraZeneca AB - Sweden; Treatment of ovarian cancer

CHMP rapporteur: Alexandre Moreau

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. COMP Strategic Review & Learning meeting, 26-28 March 2018, The Hague, The Netherlands

Action: For information

Document(s) tabled:

Invitation COMP Strategic Review and Learning Meeting 26-28 March 2018

7.1.2. Protocol Assistance Working Group (PAWG)

Proposed meeting time on 5 December 2017 at 13:00

Document(s) tabled:

PAWG draft agenda for 5 December 2017 meeting

PAWG draft minutes for 30 October 2017 meeting

7.1.3. Non-Clinical Working Group

Proposed meeting time on 10 November 2017 at 10:00

7.1.4. Condition Working Group

Proposed meeting time on 23 November 2017 at 09:30

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 November 2017

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

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

PCWP/HCPWP joint meeting – 20 September 2017

Action: For information

Document(s) tabled:

Minutes of the PCWP/HCPWP joint meeting held on 20 Sep 2017 (EMA/626905/2017)

Report of the Information session on antimicrobial resistance held on 19 Sep 2017

Draft Agenda - Training session for patients, consumers and healthcare professionals interested in EMA activities (21 Nov) - (EMA/662990/2017)

Agenda of the PCWP meeting with all eligible organisations (22 Nov) (EMA/663268/2017)

7.3.2. Scientific Advice Working Party (SAWP)

COMP: Bruno Sepodes

Scope: Re-examination of SAWP composition - call for interest to become one of the COMP representatives in the SAWP.

Action: For adoption

Document(s) tabled:
List of volunteers

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

Action: For information

Document(s) tabled:
COMP Work Plan 2018

7.8. Planning and reporting

7.8.1. List of all applications submitted/expected and the COMP coordinatorship distribution of valid applications submitted in 2017

Action: For information

7.8.2. Overview of orphan marketing authorisations/applications

Action: For information

8. Any other business

8.1. Preparedness of the system and capacity increase

Action: For discussion

8.2. S-REPS: a new way of supporting COMP procedures with a CRM (Customer Relationship Management software)

Action: For information

8.3. User manual on COMP/EMA external representation

Action: For information

8.4. COMP Workshop on Prevalence

The workshop will take place on 4 December 2017 at the EMA.

Action: For information

Document(s) tabled:
Draft agenda

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/