12 January 2018
EMA/COMP/818236/2017
Inspections, Human Medicines Pharmacovigilance and Committees

Committee for Orphan Medicinal Products (COMP)

Draft agenda for the meeting on 16-18 January 2018

Chair: Bruno Sepodes – Vice-Chair: Lesley Greene

16 January 2018, 09:00-19:30, room 2F

17 January 2018, 08:30-19:30, room 2F

18 January 2018, 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).

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

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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 16-18 January 2018. See January 2018 COMP minutes (to be published post February 2018 COMP meeting).

1.2. Adoption of agenda

COMP agenda for 16-18 January 2018.

1.3. Adoption of the minutes

COMP minutes for 05-07 December 2017.

2. Applications for orphan medicinal product designation

2.1. For opinion

2.1.1. - EMA/OD/188/17

Treatment of chronic myeloid leukaemia

Action: For adoption, Oral explanation to be held on 16 January 2018 at 09:30

Document(s) tabled:
Draft Summary report with response to LoQs

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.

2.1.2. - EMA/OD/150/17

Treatment of graft-versus-host disease

Action: For adoption, Oral explanation to be held on 16 January 2018 at 15:30

Document(s) tabled:
Draft Summary report with response to LoQs

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

2.1.3. - EMA/OD/195/17

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

Action: For information

Document(s) tabled:
Withdrawal request of 1 January 2018

2.1.4. - EMA/OD/193/17

Treatment of acute myeloid leukaemia

Action: For adoption, Oral explanation to be held on 17 January 2018 at 11:00

Document(s) tabled:
Draft Summary report with response to LoQs

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-ß-D-arabinofuranosyl cytosine, EMEA/OD/087/07 Recombinant human histone H1.3 and recombinant human N-bis-met-histone H1.3, EMEA/OD/085/07 Azacitidine, EMEA/OD/099/07 N- (2-Amino-phenyl)-4-[(4-pyridin-3-yl-pyrimidin-2-ylamino)-methyl] benzamide, EMEA/OD/118/07 Ribonucleotide reductase R2 specific phosphorothioate oligonucleotide, EMEA/OD/015/08 Sapacitabine, EMEA/OD/048/08 Daunorubicin (liposomal), EMEA/OD/105/08 N-(5-tert-Butylisoxazol-3-yl)-N'-{4-[7-(2-(morpholin-4-yl)ethoxy) imidazo[2,1-b][1,3]benzothiazol-2-yl]phenyl}urea di-hydrochloride salt, EMEA/OD/028/09 Tosedostat, EMEA/OD/091/09 1-Cyclopropyl-3-[3-(5-morpholin-4-ylmethyl-1H-benzoimidazol-2-yl)-1H-pyrazol-4-yl]-urea, EMEA/OD/147/09 2-methoxymethyl-2-hydroxymethyl-1-azabicyclo[2,2,2]octan-3-one, EMA/OD/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-[(1S)-1-carboxylato-2-cyclohexylethyl]-β-D-galactopyranosyl}oxy)-4-({6-deoxy-α-L-galactopyranosyl}oxy)-5-ethyl-cyclohexan-1-yl-(38-oxo-2,5,8,11,14,17,20,23,26,29,32,35-dodecaoxa-39-azahentetracontan-41-yl) carboxamide, EMA/OD/040/17 Entospletinib, EMA/OD/101/17 Pracinostat

Designations withdrawn: EMEA/OD/065/02 2-chloro-9-[2-deoxy-2-fluoro-ß-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 lg-like receptors (1-7 F9), EMEA/OD/020/06 Lestaurtinib, EMEA/OD/024/07 Arsenic trioxide, EMEA/OD/069/07 Amonafide L-malate, EMEA/OD/060/08 2-[[3-({4-[(5-{2-[(3-Fluorophenyl)amino]-2-oxoethyl}-1H-pyrazol-3-yl)amino]-quinazolin-7-yl}oxy)propyl](ethyl)amino]ethyl dihydrogen phosphate trihydrate, EMEA/OD/118/08 Lintuzumab, EMEA/OD/090/08 Allogeneic ex vivo expanded umbilical cord blood cells, EMEA/OD/016/09 26 base single stranded phosphodiester DNA oligonucleotide, EMEA/OD/132/09 (1S, 2S, 3R, 4R)-3-(5-Fluoro-2-(3-methyl-4-(4-methylpiperazin-1-yl)-phenylamino)-pyrimidin-4-ylamino)-bicyclo[2.2.1]hept-5-ene-2-carboxamide benzoate), EMA/OD/023/10 1-[2-(Benzo[1,2,5]thiadiazol-5-ylamino)-6-(2,6-dichloro-phenyl)-pyrido[2,3-d]pyrimidin-7-yl]-3-tert-butyl-urea, EMA/OD/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

2.1.5. - EMA/OD/171/17

Treatment of short bowel syndrome

Action: For information

Document(s) tabled:
Withdrawal request of 19 December 2017

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

2.1.6. - EMA/OD/305/16

Treatment of ovarian cancer

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

Document(s) tabled:
Draft Summary report with response to LoQs

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-hydroxycamptothecine 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 Epothilone B, EMEA/OD/016/03 Murine anti-idiotypic antibody against OC125 antibody against CA125 antigen, EMEA/OD/071/09 Anti-EphA2 monoclonal antibody conjugated to maleimidocaproyl monomethylauristatin phenylalanine, EMA/OD/014/10 Pyr-His-Trp-Ser-Tyr-D-Lys(doxorubicinylglutarate)-Leu-Arg-Pro-Gly-NH2, acetate salt, EMA/OD/094/11 Vincaleukoblastin-23-oic acid, O4-deacetyl-2-[(2-mercaptoethoxy)carbonyl]hydrazide, disulfide with N-[4-[[(2-amino-3,4-dihydro-4-oxo-6-pteridinyl)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

2.1.7. - EMAOD/170/17

Treatment of soft tissue sarcoma

Action: For adoption, Oral explanation to be held on 17 January 2018 at 14:30

Document(s) tabled:
Draft Summary report with response to LoQs

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-epoxypyrido[2,1-c][1,4]oxazacyclohentriacontin-3-yl]propyl}-2-methoxy-cyclohexyldimethyl-phosphinate, EMEA/OD/071/05 Brostallicin, EMEA/OD/083/06

Fenretinide, EMEA/OD/044/08 Palifosfamide, EMA/OD/141/10 Ombrabulin, EMA/OD/110/11 (1-methyl-2-nitro-1H-imidazole-5-yl)methyl N,N'-bis(2-bromoethyl) diamidophosphate

2.1.8. - EMA/OD/189/17

Treatment of haemophilia A

Action: For adoption, Oral explanation to be held on 17 January 2018 at 15:30

Document(s) tabled:
Draft Summary report with response to LoQs

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

2.1.9. - EMA/OD/190/17

Treatment of haemophilia B

Action: For information

Document(s) tabled:
Withdrawal request of 2 January 2018

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

2.1.10. - EMA/OD/173/17

Treatment of cystic fibrosis

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

Document(s) tabled:
Draft Summary report with response to LoQs

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, phospatidylinositol and cholesterol, EMA/OD/046/11 Cysteamine, EMA/OD/058/12 Alpha-1 proteinase inhibitor (for inhalation use), EMA/OD/005/13 Recombinant human CXCL8 mutant, EMA/OD/017/13 4,6,4'-trymethylangelicin, EMA/OD/096/13 Antisense oligonucleotide targeting the F508delta mutation of CFTR, EMA/OD/095/13 Nitric oxide, EMA/OD/159/13 Cysteamine, EMA/OD/156/13 11-(4-Dimethylamino-3-hydroxy-6-methyl-tetrahydro-pyran-2-yloxy)-2-ethyl-3,4,10-trihydroxy-3,5,6,8,10,12,14-heptamethyl-1-oxa-6-aza-cyclopentadecane-13,15-dione, EMA/OD/036/14 Nitric oxide, EMA/OD/013/14 Plasmid DNA encoding the human cystic fibrosis transmembrane conductance regulator gene complexed with a non-viral, cationic lipid based gene transfer agent, EMA/OD/002/14 1-(2,2-difluoro-1,3-benzodioxol-5-yl)-N-{1-[(2R)-2,3-dihydroxypropyl]-6-fluoro-2-(1-hydroxy-2-methylpropan-2-yl)-1Hindol-5-yl}cyclopropanecarboxamide, EMA/OD/131/14 4-[[(1S,4S)-5-[[4-[4-(Oxazol-2-yl)phenoxy]phenyl]methyl]-2,5-diazabicyclo[2.2.1]hept-2-yl]methyl]benzoic acid, EMA/OD/018/15 2-(7-ethoxy-4-(3-fluorophenyl)-1-oxophthalazin-2(1H)-yl)-N-methyl-N-(2-methylbenzo[d]oxazol-6-yl)acetamide, EMA/OD/319/14 Nitric oxide, EMA/OD/068/15 Fixed-dose combination of fosfomycin disodium and tobramycin, EMA/OD/061/15 Recombinant human acid ceramidase, 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

2.1.11. - EMA/OD/178/17

Treatment of Stargardt's disease

Action: For information

Document(s) tabled:
Withdrawal request of 21 December 2017

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

2.1.12. - EMA/OD/180/17

Treatment of acromegaly

Action: For information

Document(s) tabled:
Withdrawal request of 4 January 2018

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

2.1.13. - EMA/OD/198/17

Treatment of glioma

Action: For adoption, Oral explanation to be held on 18 January 2018 at 11:00

Document(s) tabled:
Draft Summary report with response to LoQs

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 Gimatecan, 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 circulary-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-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 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-(triflurомethyl)benzylazanediyl)bis(methylene) diquinolin-8-ol, EMA/OD/159/14 Chloroquine, EMA/OD/176/14 Adenovirus serotype 5 containing partial E1A deletion and an integrin-binding domain, EMA/OD/251/14 Recombinant human glutamate oxaloacetate transaminase 1, 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

diptheria 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] diazepin-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

2.1.14. - EMA/OD/185/17

Treatment of glioma

Action: For adoption, Oral explanation to be held on 18 January 2018 at 11:00

Document(s) tabled:
Draft Summary report with response to LoQs

Notes: There have been 41 designations for this condition: See 2.1.13.

2.1.15. - EMA/OD/191/17

Treatment in cardiopulmonary by-pass

Action: For adoption, Oral explanation to be held on 18 January 2018 at 12:00

Document(s) tabled:
Draft Summary report with response to LoQs

2.1.16. - EMA/OD/176/17

Treatment of Myasthenia Gravis

Action: For adoption, Oral explanation to be held on 18 January 2018 at 14:30

Document(s) tabled:
Draft Summary report with response to LoQs

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

2.1.17. - EMA/OD/312/16

Treatment of hepatocellular carcinoma

Action: For adoption

Document(s) tabled:
Draft Summary report with response to LoQs

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"-fluorenylideneaminooxy)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. For discussion / preparation for an opinion

2.2.1. - EMA/OD/208/17

Treatment of C3 glomerulopathy

Action: For discussion/adoption

Document(s) tabled:
Draft Summary report

Notes: There have been 2 designations for this condition: EMA/OD/104/15 S3,S13-cyclo(D-tyrolsyl-L-isoleucyl-L-cysteinyl-L-valyl-1-methyl-L-tryptophyl-L-glutaminyl-L-aspartyl-L-tryptophyl-N-methyl-L-glycyl-L-alanyl-L-histidyl-L-arginyl-L-cysteinyl-N-methyl-L-isoleucinamide), EMA/OD/028/17 Avacopan

2.2.2. - EMA/OD/203/17

Treatment of cystic fibrosis

Action: For discussion/adoption

Document(s) tabled:
Draft Summary report

Notes: There have been 39 designations for this condition: See 2.1.10.

2.2.3. - EMA/OD/202/17

Treatment of soft tissue sarcoma

Action: For discussion/adoption

Document(s) tabled:
Draft Summary report

Notes: There have been 16 designations for this condition: See 2.1.7.

2.2.4. - EMA/OD/197/17

Treatment of retinitis pigmentosa

Action: For discussion/adoption

Document(s) tabled:
Draft Summary report

Notes: There have been 22 designations for this condition: EMEA/OD/057/06 4,7,10,13,16,19-Docosahexaenoic acid, EMEA/OD/043/07 Adenovirus associated viral vector serotype 4 containing the human RPE65 gene, EMEA/OD/087/08 Recombinant human proinsulin, EMA/OD/162/10 9-cis-Retinyl acetate, EMA/OD/159/11 Encapsulated human retinal pigment epithelial cell line transfected with plasmid vector expressing human ciliary neurotrophic factor, EMA/OD/006/12 Recombinant human methionine proinsulin, EMA/OD/025/13 Expanded human allogeneic neural retinal progenitor cells extracted from neural retina, EMA/OD/015/13 Recombinant human nerve growth factor, EMA/OD/031/13 Adenovirus associated viral vector serotype 5 containing the human pde6β gene, EMA/OD/289/14 Sodium 3-[(4aR,6R,7R,7aS)-7-hydroxy-2-oxido-2-sulfanylidene-4a,6,7,7a-tetrahydro-4H-furo[3,2-d][1,3,2]dioxaphosphinin-6-yl]-2-bromo-6-phenyl-5H-imidazo[1,2-a]purin-9-one, EMA/OD/271/14 Myriocin, EMA/OD/327/14 Recombinant human mesencephalic astrocyte-derived neurotrophic factor, EMA/OD/040/15 Adenovirus-associated viral vector serotype 2 containing the human RPE65 gene, EMA/OD/158/16 Adeno-associated viral vector serotype 2/2 containing a gene encoding the channelrhodopsin-2 protein, EMA/OD/213/15 Allogeneic fetal human retinal progenitor cells expanded ex vivo, EMA/OD/028/16 Adeno-associated viral vector serotype 2.7m8 containing the ChrimsonR-tdTomato gene, EMA/OD/102/16 Adenovirus associated viral vector serotype 5 containing the human RPGR gene, EMA/OD/146/16 Adeno-associated viral vector serotype 5 containing the human RLBP1 gene, EMA/OD/165/16 Adeno-associated viral vector serotype 8 encoding engineered rhodopsin DNA-binding repressor and human rhodopsin expression cassettes, EMA/OD/280/16 Antisense oligonucleotide targeting the USH2A gene, EMA/OD/086/17 Recombinant truncated N-terminal fragment of human lens epithelium derived growth factor, EMA/OD/094/17 Antisense oligonucleotide targeting exon 13 in the USH2A gene

Designations withdrawn: EMEA/OD/075/07 Recombinant human rod-derived cone viability factor, EMEA/OD/106/07 Allogeneic human umbilical cord tissue-derived cells, EMA/OD/021/12 17-(Dimethylaminoethylamino)-17-demethoxygeldanamycin (after administration of adeno-associated viral vector encoding an inducible short hairpin RNA targeting claudin-5), EMA/OD/135/12 Adeno-associated viral vector encoding an inducible short hairpin RNA targeting claudin-5 (prior to administration of 17-dimethylaminoethylamino-17-demethocygeldanamycin), EMA/OD/067/13 Unoprostone isopropyl, EMA/OD/208/15 4-[(2E)-1-oxo-3-(2,6,6-trimethyl-1-cyclohexen-1-yl)-2-propen-1-yl]-1-piperazinecarboxamide

2.2.5. - EMA/OD/220/17

Treatment of Retinitis Pigmentosa

Action: For discussion/adoption

Document(s) tabled:
Draft Summary report

Notes: There have been 22 designations for this condition: See 2.2.4.

2.2.6. - EMA/OD/204/17

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: See 2.1.2.

2.2.7. - EMA/OD/215/17

Treatment of Fragile X Syndrome

Action: For discussion/adoption

Document(s) tabled:
Draft Summary report

Notes: There have been 9 designations for this condition: EMA/OD/144/10 R-baclofen, EMA/OD/059/12 Mavoglurant, EMA/OD/105/14 (3S)-(+)-(5-chloro-2-methoxyphenyl)-1,3-dihydro-3-fluoro-6-(trifluoromethyl)-2H-indol-2-one, EMA/OD/137/14 Acamprosate calcium, EMA/OD/253/14 Tideglusib, EMA/OD/055/15 Glycyl-L-2-methylprolyl-L-glutamic acid, EMA/OD/080/15 Alpha-tocopherol and ascorbic acid, EMA/OD/293/16 N-[(1R)-1-phenylethyl]-6-{1H-pyrazolo[3,4-d]pyrimidin-4-yl}quinazolin-2-amine, EMA/OD/034/16 Pyridoxine and L-pyroglutamic acid

2.2.8. - EMA/OD/062/17

Treatment of Dravet syndrome

Action: For discussion/adoption

Document(s) tabled:
Draft Summary report

Notes: There have been 3 designations for this condition: EMA/OD/140/13 Fenfluramine hydrochloride, EMA/OD/083/14 Cannabidiol, EMA/OD/221/16 26 base synthetic single-stranded fully phosphorothioated 2'-omethyl-RNA and DNA mixmer oligonucleotide-based compound

2.2.9. - EMA/OD/219/17

Treatment of Friedreich's ataxia

Action: For discussion/adoption

Document(s) tabled:
Draft Summary report

Notes: There have been 4 designations for this condition: EMEA/OD/037/01 Idebenone, EMEA/OD/082/03 Idebenone, EMA/OD/026/10 N-(6-(2-aminophenylamino)-6-oxohexyl)-4-methylbenzamide, EMA/OD/084/11 Interferon gamma

2.2.10. - EMA/OD/172/17

Treatment of biliary tract cancer

Action: For discussion/adoption

Document(s) tabled:
Draft Summary report

Notes: There have been 4 designations for this condition: EMA/OD/199/13 (5R,5aR,8aR,9S)-9-[[4,6-O-[(R)-Ethylidene]-beta-D-glucopyranosyl]-oxy]-5-(4-({[(2,2-dimethyl-1,3-dioxolan-4-yl)methoxy]carbonyl}oxy)-3,5-dimethoxyphenyl)-5,8,8a,9-tetrahydroisobenzofuro[5,6-f][1,3]benzodioxol-6(5aH)-one, EMA/OD/305/14 5,10,15,20-tetrakis(2,6-difluoro-3-N-methylsulfamoylphenyl)bacteriochlorin, EMA/OD/245/15 (R)-6-(2-fluorophenyl)-N-(3-(2-((2-methoxyethyl)amino)ethyl)phenyl)-5,6-dihydrobenzo[h] quinazolin-2-amine dihydrochloride, EMA/OD/124/17 5-amino-1-(2-methyl-1H-benzo[d]imidazol-5-yl)-1H-pyrazol-4-yl 1H-indol-2-yl ketone mono[(S)-2-hydroxysuccinate]

2.2.11. - EMA/OD/210/17

Treatment of NTRK-fusion non-small-cell lung cancer

Action: For discussion/adoption

Document(s) tabled:
Draft Summary report

2.2.12. - EMA/OD/212/17

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

2.2.13. - EMA/OD/209/17

Treatment of biliary tract cancer

Action: For discussion/adoption

Document(s) tabled:
Draft Summary report

Notes: There have been 4 designations for this condition: See 2.2.10.

2.2.14. - EMA/OD/213/17

Treatment of salivary gland cancer

Action: For discussion/adoption

Document(s) tabled:
Draft Summary report

2.2.15. - EMA/OD/211/17

Treatment of pancreatic cancer

Action: For discussion/adoption

Document(s) tabled:
Draft Summary report

Notes: There have been 37 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-GNNDESNISFKEK, EMEA/OD/030/09 Trabedersen, EMEA/OD/105/09 Brivudine, EMEA/OD/069/09 N-[(2S)-2,3-dihydroxypropyl]-3-[(2-fluoro-4-iodophenyl)amino]isonicotinamide hydrochloride, EMEA/OD/063/09 Masitinib mesilate, EMA/OD/135/10 Glufosfamide, EMA/OD/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, 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 inlB 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/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

2.2.16. - EMA/OD/174/17

Treatment of amyotrophic lateral sclerosis

Action: For discussion/adoption

Document(s) tabled:
Draft Summary report

Notes: There have been 22 designations for this condition: EMEA/OD/053/06 Arimoclomol, EMEA/OD/102/07 Filgrastim, EMEA/OD/096/08 (6R)-4,5,6,7-tetrahydro-N6-propyl-2,6-benzothiazole-diamine dihydrochloride monohydrate, EMEA/OD/108/09 Recombinant human vascular endothelial growth factor, EMA/OD/043/11 Smilagenin, EMA/OD/106/11 S[+] apomorphine, EMA/OD/138/11 6-ethynyl-1-(pentan-3-yl)-1H-imidazo[4,5-b]pyrazin-2(3H)-one, EMA/OD/011/13 Autologous bone marrow-derived mesenchymal stromal cells secreting neurotrophic factors, EMA/OD/023/13 Sodium chlorite, EMA/OD/044/13 Allogeneic motor neuron progenitor cells derived from human embryonic stem cells, EMA/OD/184/14 Edaravone, EMA/OD/283/14 Enoxacin, EMA/OD/032/15 Edaravone, EMA/OD/051/15 Hydrocinnamate-[Orn-Pro-dCha-Trp-Arg]acetate, EMA/OD/011/16 H-Phe-Ser-Arg-Tyr-Ala-

Arg-OH-acetate, EMA/OD/241/15 Recombinant human cerebral dopamine neurotrophic factor, EMA/OD/081/16 Masitinib mesilate, EMA/OD/120/16 Synthetic ribonucleic acid oligonucleotide directed against superoxide dismutase 1 messenger ribonucleic acid, EMA/OD/182/16 Ibudilast, EMA/OD/242/16 Tauroursodeoxycholic acid, EMA/OD/030/17 Recombinant human antibody directed against misfolded human superoxide dismutase 1, EMA/OD/136/17 (R)-troloxamide quinone
Designations withdrawn: EMEA/OD/029/00 Xaliproden hydrochloride, EMEA/OD/030/06 Cholest-4-en-3-one, oxime, EMEA/OD/125/07 Sarsasapogenin, EMEA/OD/012/09 Talampanel, EMA/OD/060/10 Recombinant humanised monoclonal antibody to human Nogo-A protein of the IgG1/kappa class

2.2.17. - EMA/OD/199/17

Treatment of pulmonary arterial hypertension

Action: For discussion/adoption

Document(s) tabled:
Draft Summary report

Notes: There have been 6 designations for this condition: EMEA/OD/018/08 Beraprost sodium, EMA/OD/023/11 Macitentan, EMA/OD/111/11 Sodium nitrite, EMA/OD/179/15 Ubenimex, EMA/OD/299/16 (S)-8-{2-amino-6-[1-(5-chloro-biphenyl-2-yl)-(R)-2,2,2-trifluoro-ethoxy]-pyrimidin-4-yl}-2,8-diaza-spiro[4.5]decane-3-carboxylic acid ethyl ester), EMA/OD/099/17 Tacrolimus

2.2.18. - EMA/OD/181/17

Treatment of non-traumatic subarachnoid haemorrhage

Action: For discussion/adoption

Document(s) tabled:
Draft Summary report

Notes: There has been 1 designation for this condition: EMA/OD/120/17 1,4-diamino-2,3-dicyano-1,4-bis[2-aminophenylthio] butadiene

2.2.19. - EMA/OD/206/17

Treatment of naevoid basal cell carcinoma syndrome (Gorlin syndrome)

Action: For discussion/adoption

Document(s) tabled:
Draft Summary report

Notes: Designation withdrawn: EMEA/OD/048/09 N-[6-(cis-2,6-DimItraconazoleethylmorpholin-4-yl)pyridine-3-yl]-2-methyl-4'-(trifluoromethoxy)[1,1'-biphenyl]-3-carboxamide

2.2.20. - EMA/OD/201/17

Treatment of pyridox(am)ine 5'-phosphate oxidase deficiency

Action: For discussion/adoption

Document(s) tabled:
Draft Summary report

Notes: There has been 1 designation for this condition: EMA/OD/104/14 Pyridoxal 5'-phosphate

2.2.21. - EMA/OD/200/17

Treatment of primary immunoglobulin A nephropathy

Action: For discussion/adoption

Document(s) tabled:
Draft Summary report

Notes: There has been 1 designation for this condition: EMA/OD/139/16 Budesonide

2.2.22. - EMA/OD/221/17

Treatment of acute radiation syndrome

Action: For discussion/adoption

Document(s) tabled:
Draft Summary report

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

2.2.23. - EMA/OD/205/17

Treatment of activated phosphoinositide 3-kinase delta syndrome

Action: For discussion/adoption

Document(s) tabled:
Draft Summary report

2.2.24. - EMA/OD/222/17

Treatment of follicular lymphoma

Action: For discussion/adoption

Document(s) tabled:
Draft Summary report

Notes: There have been 9 designations for this condition: EMEA/OD/040/06 Autologous tumor-derived immunoglobulin idiotype coupled to keyhole limpet haemocyanin, EMEA/OD/065/04 Recombinant hisitidine-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-tetulomab, 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
Designations withdrawn: EMEA/OD/061/02 Iodine (131I) tositumomab, EMEA/OD/079/02 Tositumomab, EMA/OD/053/13 Idelalisib

2.2.25. - EMA/OD/217/17

Treatment of diffuse large B-cell lymphoma

Action: For discussion/adoption

Document(s) tabled:
Draft Summary report

Notes: There have been 12 designations for this condition: EMEA/OD/091/08 Recombinant hisitidine-tagged idiotype immunoglobulin Fab fragment of clonal B-cell receptors, EMA/OD/160/10 Lenalidomide, EMA/OD/116/13 Ibrutinib, EMA/OD/092/14 obinutuzumab, EMA/OD/215/14 Humanised Fc engineered monoclonal antibody against CD19, EMA/OD/005/15 Humanised anti-CD37 monoclonal antibody conjugated to maytansinoid DM1, EMA/OD/016/16 3-(5-amino-2-methyl-4-oxoquinazolin-3(4H)-yl)piperidine-2,6-dione hydrochloride, EMA/OD/087/16 autologous T cells transduced with lentiviral vector containing a chimeric antigen receptor directed against CD19, EMA/OD/162/16 Valproic acid, EMA/OD/122/16 Venetoclax, EMA/OD/229/16 5-(4,6-dimorpholino-1,3,5-triazin-2-yl)-4-(trifluoromethyl)pyridin-2-amine, EMA/OD/045/17 Autologous CD4+ and CD8+ T cells expressing a CD19-specific chimeric antigen receptor
Designations withdrawn: EMEA/OD/126/09 Pixantrone dimaleate, EMA/OD/084/15 2'-deoxyguanosylyl-(3',5'-phosphoryl)-2'-deoxythymidylyl-(3',5'-phosphoryl)- 2'-deoxyguanosylyl-(3',5'-phosphoryl)-2'-deoxycytidylyl-(3',5'-phosphoryl)-2'-deoxycytidylyl -(3',5'-phosphoryl)-2'-deoxycytidylyl-(3',5'-phosphoryl)-2'-deoxyguanosylyl-(3',5'-phosphoryl)-2'-deoxycytidilyl-(3',5'-phosphoryl)-2'-deoxycytidylyl-(3',5'-phosphoryl)-2'-deoxycytidylyl-(3',5'-phosphoryl)-2'-deoxycytidylyl-(3',5'-phosphoryl)-2'-deoxythymidylyl-(3',5'-phosphoryl)-2'-deoxyadenosyl-(3',5'-phosphoryl)-2'-deoxycytidylyl-(3',5'-phosphoryl)-2'-deoxyguanosylyl-(3',5'-phosphoryl)-2'-deoxycytidylyl-(3',5'-phosphoryl)-2'-deoxyguanosylyl-(3',5'-phosphoryl)-2'-deoxycytidylyl-(3',5'-phosphoryl)-2'-deoxyadenosyl-(3',5'-phosphoryl)-2'-deoxycytidylyl-(3',5'-phosphoryl)-2'-deoxyguanosylyl-2'-deoxycytidylyl-(3',5'-phosphoryl)-2'-deoxyadenosyl-(3',5'-phosphoryl)-2'-deoxycytidine, sodium salt

2.2.26. - EMA/OD/216/17

Treatment of soft tissue sarcoma

Action: For discussion/adoption

Document(s) tabled:
Draft Summary report

Notes: There have been 16 designations for this condition: See 2.1.7.

2.3. Revision of the COMP opinions

None

2.4. Amendment of existing orphan designations

None

2.5. Appeal

2.5.1. Melatonin – EMA/OD/039/17

Therapicon Srl; Treatment of partial deep dermal and full thickness burns

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

Document(s) tabled:
Revised draft Summary report
Sponsor's grounds for appeal

2.6. Nominations

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

Action: For adoption

Document(s) tabled:
OMPD applications - appointment of coord. at the 16-18 January 2018 COMP meeting

2.7. Evaluation on-going

Twenty four 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 acute myeloid leukaemia

Action: For adoption

3.1.2. -

Treatment of Niemann-Pick disease, type C

Action: For adoption

3.1.3. -

Treatment of mucopolysaccharidosis type I

Action: For adoption

3.1.4. -

TKI inhibitor for treatment of gastrointestinal stromal tumors

Action: For adoption

3.1.5. -

Treatment of cutaneous T-cell lymphoma

Action: For adoption

3.1.6. -

Treatment of diffuse large B-cell lymphoma

Action: For adoption

3.1.7. -

Treatment of multiple myeloma

Action: For adoption

3.1.8. -

Treatment of glioma

Action: For adoption

3.1.9. -

Treatment of multiple myeloma

Action: For adoption

3.1.10. -

Treatment of paroxysmal nocturnal haemoglobinuria

Action: For adoption

3.2. Finalised letters

3.2.1. -

Treatment of spinal muscular atrophy

Action: For information

3.2.2. -

Treatment of plasma cell myeloma

Action: For information

3.2.3. -

Treatment of sickle cell disease

Action: For information

3.2.4. -

Treatment of ornithine transcarbamylase deficiency

Action: For information

3.2.5. -

Treatment of Lennox-Gastaut syndrome

Action: For information

3.2.6. -

Treatment of mantle cell lymphoma

Action: For information

3.2.7. -

Treatment of myelodysplastic syndromes

Action: For information

3.2.8. -

Treatment of Leber's hereditary optic neuropathy

Action: For information

3.3. New requests

3.3.1. -

Treatment of adenosine deaminase-deficient-severe combined immunodeficiency

Action: For information

3.3.2. -

Treatment of acute hepatic porphyria

Action: For information

3.3.3. -

Treatment of adrenoleukodystrophy

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

None

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

4.2.1. - 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

4.2.2. - metreleptin – EMEA/H/C/004218

Aegerion Pharmaceuticals Limited;

a) Treatment of familial partial lipodystrophy EMA/OD/033/12, EU/3/12/1022

b) Treatment of Barraquer-Simons syndrome EMA/OD/034/12, EU/3/12/1023

c) Treatment of Lawrence syndrome EMA/OD/035/12, EU/3/12/1024

d) Treatment of Berardinelli-Seip syndrome EMA/OD/036/12, EU/3/12/1025

Action: For discussion

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

4.3. Appeal

4.3.1. Alofisel – DARVADSTROCEL – 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 16 January 2018 at 14:30

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

4.3.2. Verkazia - ciclosporin – EMEA/H/C/004411, EMEA/OD/106/05, EU/3/06/360

Santen Oy; Treatment of vernal keratoconjunctivitis

Action: For discussion

Document(s) tabled:
EC letter

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

4.6. Orphan Maintenance Reports

Action: For information

Document(s) tabled:

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

5.1. After adoption of CHMP opinion

None

5.2. Prior to adoption of CHMP opinion

5.2.1. 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 information

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

5.2.2. Darzalex - Daratumumab – EMEA/H/C/004077/II/0011, EMA/OD/038/13, EU/3/13/1153

Janssen-Cilag International N.V.; Treatment of plasma cell myeloma

CHMP rapporteur: Sinan B. Sarac; CHMP co-rapporteur: Jorge Camarero Jiménez

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 16 January 2018 at 13:00

Document(s) tabled:
PAWG draft agenda for 16 January 2018 meeting
PAWG draft minutes for 5 December 2017 meeting

7.1.3. Non-Clinical Working Group

Proposed meeting time on 17 January 2018 at 08:30

7.1.4. Condition Working Group

Proposed meeting time on 18 January 2018 at 08:30

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 December 2017

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

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

Action: For information

Document(s) tabled:
Meeting Summary PCWP meeting with all eligible organisations – 22 Nov

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

None

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 coordinatorship 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. Preparedness of the system and capacity increase

Action: For information

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

Action: For information

8.3. EMA Business Pipeline activity and Horizon scanning

Action: For information

Document tabled:
Upcoming Q4/2017 Update of the Business Pipeline report for the human scientific committees

8.4. PRIME products DASHBOARD

Action: For discussion

8.5. Concepts of significant benefit and relative effectiveness, EMA-EUnetHTA work plan Jan 2017 – May 2020

Action: For discussion

Document tabled:
SB versus REA research proposal EMA-EUnetHTA_for_COMP_meeting
Project_proposal_draft

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/