12 February 2018
EMA/COMP/40855/2018
Inspections, Human Medicines Pharmacovigilance and Committees

Committee for Orphan Medicinal Products (COMP)

Draft agenda for the meeting on 13-15 February 2018

Chair: Bruno Sepodes – Vice-Chair: Lesley Greene

13 February 2018, 09:00-19:30, room 2F

14 February 2018, 08:30-19:30, room 2F

15 February 2018, 08:30-13:00, room 2F

Health and safety information

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

Disclaimers

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

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

Note on access to documents

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

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

Table of contents

1. Introduction

1.1. Welcome and declarations of interest of members and experts

Pre-meeting list of participants and restrictions in relation to declarations of interests applicable to the items of the agenda for the COMP plenary session to be held 13-15 February 2018. See February 2018 COMP minutes (to be published post March 2018 COMP meeting).

1.2. Adoption of agenda

COMP agenda for 13-15 February 2018.

1.3. Adoption of the minutes

COMP minutes for 16-18 January 2018.

2. Applications for orphan medicinal product designation

2.1. For opinion

2.1.1. - EMA/OD/062/17

Treatment of Dravet syndrome

Action: For adoption, Oral explanation to be held on 13 February 2018 at 14:00

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

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

2.1.2. - EMA/OD/204/17

Treatment of graft-versus-host disease

Action: For adoption, Oral explanation to be held on 13 February 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/181/17

Treatment of non-traumatic subarachnoid haemorrhage

Action: For adoption, Oral explanation to be held on 13 February 2018 at 17:00

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

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

2.1.4. - EMA/OD/219/17

Treatment of Friedreich's ataxia

Action: For adoption, Oral explanation to be held on 14 February 2018 at 09:30

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

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

2.1.5. - EMA/OD/211/17

Treatment of pancreatic cancer

Action: For adoption, Oral explanation to be held on 14 February 2018 at 11:00

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

Notes: There have been 36 designations for this condition: EMEA/OD/055/02 G17(9) gastrin-Diphtheria Toxoid Conjugate, EMEA/OD/068/02 Rubitecan, EMEA/OD/009/05 Bovine bile extract, EMEA/OD/028/05 4-imino-1, 3-diazobicyclo-[3.1.0]-hexan-2-one, EMEA/OD/063/06 Paclitaxel (liposomal), EMEA/OD/026/06 Human telomerase reverse transcriptase peptide (611-626), EMEA/OD/103/06 Cisplatin (liposomal), EMEA/OD/100/08 L-asparaginase encapsulated in erythrocytes, EMEA/OD/006/08 Nimotuzumab, EMEA/OD/080/08 Yttrium (90Y)-DOTA-radiolabelled humanized monoclonal antibody against mucin 1, EMEA/OD/101/08 S-[2,3-bispalmitoyloxy-(2R)-propyl]-cysteinyl-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/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 k-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/065/12 Mixture of two allogeneic human pancreatic cancer cell lines stably transduced with a retroviral vector encoding the murine alpha-(1,3)-galactosyltransferase gene, EMA/OD/145/12 Recombinant human monoclonal antibody of the IgG1 kappa class against prostate stem cell antigen, EMA/OD/037/13 (1-methyl-2-nitro-1H-imidazole-5-yl)methyl N,N'-bis(2-bromoethyl)diamidophosphate

2.1.6. - EMA/OD/213/17

Treatment of salivary gland cancer

Action: For adoption, Oral explanation to be held on 14 February 2018 at 11:00

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

2.1.7. - EMA/OD/209/17

Treatment of biliary tract cancer

Action: For adoption, Oral explanation to be held on 14 February 2018 at 11:00

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

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]

2.1.8. - EMA/OD/212/17

Treatment of papillary thyroid cancer

Action: For adoption, Oral explanation to be held on 14 February 2018 at 11:00

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

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

2.1.9. - EMA/OD/210/17

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

Action: For adoption, Oral explanation to be held on 14 February 2018 at 11:00

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

2.1.10. - EMA/OD/206/17

Treatment of naevoid basal cell carcinoma syndrome (Gorlin syndrome)

Action: For adoption, Oral explanation to be held on 13 February 2018 at 12:00

Document(s) tabled:

Draft Summary report with response to LoQs

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

2.1.11. - EMA/OD/222/17

Treatment of follicular lymphoma

Action: For adoption, Oral explanation to be held on 14 February 2018 at 15:30

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

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

2.1.12. - EMA/OD/216/17

Treatment of soft tissue sarcoma

Action: For adoption, Oral explanation to be held on 14 February 2018 at 15:30

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

2.1.13. - EMA/OD/217/17

Treatment of diffuse large B-cell lymphoma

Action: For adoption, Oral explanation to be held on 14 February 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/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

2.1.14. - EMA/OD/208/17

Treatment of C3 glomerulopathy

Action: For adoption

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

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

2.1.15. - EMA/OD/172/17

Treatment of biliary tract cancer

Action: For adoption

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

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. For discussion / preparation for an opinion

2.2.1. - EMA/OD/246/17

Treatment of ovarian cancer

Action: For discussion/adoption

Document(s) tabled:
Draft Summary report

Notes: There have been 30 designations for this condition: EMEA/OD/019/02 Oregovomab, EMEA/OD/061/06 Paclitaxel (micellar), EMEA/OD/080/03 Anti-epithelial cell adhesion molecule/anti-CD3 monoclonal antibody, EMEA/OD/044/03 Trabectedin, EMEA/OD/065/05 Imexon, EMEA/OD/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

2.2.2. - EMA/OD/081/17

Treatment of intestinal failure-associated liver disease

Action: For discussion/adoption

Document(s) tabled:
Draft Summary report

2.2.3. - EMA/OD/235/17

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 bicolour 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

2.2.4. - EMA/OD/245/17

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

2.2.5. - EMA/OD/233/17

Treatment of snakebite envenomation

Action: For discussion/adoption

Document(s) tabled:
Draft Summary report

Notes: There has been 1 designation for this condition: EMA/OD/062/15 Ovine-specific immunoglobulin (Fab) fragments raised against Vipera berus venom

2.2.6. - EMA/OD/218/17

Treatment of Neuronal Ceroid Lipofuscinosis

Action: For discussion/adoption

Document(s) tabled:
Draft Summary report

Notes: There has been 1 designation for this condition: EMA/OD/195/16 Recombinant self-complementary adeno-associated viral vector serotype 9 encoding the human CLN3gene

2.2.7. - EMA/OD/244/17

Treatment of Epidermolysis Bullosa

Action: For discussion/adoption

Document(s) tabled:
Draft Summary report

Notes: There have been 14 designations for this condition: EMEA/OD/111/05 Bilayer engineered skin composed of keratinocytes from the patient (autologous) and fibroblasts from a donor (allogeneic) embedded in a plasma matrix, EMEA/OD/061/09 Allogeneic human dermal fibroblasts, EMA/OD/120/10 Dry extract from birch bark (DER 0.1-0.2:1), extraction solvent n-heptane 95% (V/V), EMA/OD/145/13 Allantoin, EMA/OD/149/13 Diacerein, EMA/OD/201/13 Recombinant human alpha 1 chain homotrimer of type VII collagen, EMA/OD/197/14 Allogeneic adipose-derived adult mesenchymal stem cells contained in a fibrin-based bioengineered dermis, EMA/OD/218/15 Autologous dermal fibroblasts genetically modified ex vivo with a lentiviral vector containing the human COL7A1 gene, EMA/OD/299/14 Ex-vivo-expanded autologous human keratinocytes containing epidermal stem cells transduced with a COL17A1-encoding retroviral vector, EMA/OD/297/14 Ex-vivo-expanded autologous human keratinocytes containing epidermal stem cells transduced with a LAMB3-encoding retroviral vector, EMA/OD/188/15 Ex-vivo-expanded autologous fibroblasts transduced with lentiviral vector containing the COL7A1 gene, EMA/OD/283/16 Ex-vivo-expanded autologous keratinocytes transduced with retroviral vector containing the COL7A1 gene, EMA/OD/031/17 Asp-Arg-Val-Tyr-Ile-His-Pro, EMA/OD/140/17 Antisense oligonucleotide targeting exon 73 in the COL7A1 gene Designation withdrawn: EMA/OD/172/10 Human dermal fibroblasts cultured on a bioresorbable polyglactin mesh

2.2.8. - EMA/OD/194/17

Treatment of adrenal insufficiency

Action: For discussion/adoption

Document(s) tabled:
Draft Summary report

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

2.2.9. - EMA/OD/214/17

Treatment of polycythemia vera

Action: For discussion/adoption

Document(s) tabled:
Draft Summary report

Notes: There have been 6 designations for this condition: EMA/OD/019/11 N-(cyanomethyl)-4-(2-{[4-(morpholin-4-yl)phenyl]amino}pyrimidin-4-yl)benzamide, dihydrochloride salt, EMA/OD/092/10 N-tert-butyl-3-[(5-methyl-2-{[4-(2-pyrrolidin-1-ylethoxy)phenyl]amino}pyrimidin-4-yl)amino] benzenesulfonamide dihydrochloride monohydrate, EMA/OD/057/10 11-(2-pyrrolidin-1-yl-ethoxy)-14,19-dioxa-5,7,26-triaza-tetracyclo[19.3.1.1(2,6).1(8,12)] heptacosa-1(25),2(26),3,5,8,10,12(27),16,21,23-decaene, EMA/OD/048/10 Pomalidomide, EMA/OD/122/10 Plitidepsin, EMA/OD/139/14 Recombinant human Pentraxin-2,

2.2.10. - EMA/OD/247/17

Treatment of Guillain-Barré syndrome

Action: For discussion/adoption

Document(s) tabled:
Draft Summary report

Notes: There has been 1 designation for this condition: EMA/OD/030/16 Recombinant protein derived from the saliva of the Ornithodoros moubata tick
Designation withdrawn: EMEA/OD/101/06 Fampridine

2.2.11. - EMA/OD/237/17

Treatment of anaplastic thyroid cancer

Action: For discussion/adoption

Document(s) tabled:
Draft Summary report

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

2.2.12. - EMA/OD/238/17

Treatment of follicular thyroid cancer

Action: For discussion/adoption

Document(s) tabled:
Draft Summary report

Notes: There have been 2 designations for this condition: EMA/OD/019/13 Lenvatinib, EMA/OD/092/13 Sorafenib tosylate

2.2.13. - EMA/OD/187/17

Treatment of ornithine transcarbamylase deficiency (OTC)

Action: For discussion/adoption

Document(s) tabled:
Draft Summary report

Notes: There have been 7 designations for this condition: EMA/OD/026/11 Heterologous human adult liver-derived stem cells, EMA/OD/097/11 Sodium phenylbutyrate, EMA/OD/053/16 Sodium benzoate, EMA/OD/310/16 Adeno-associated viral vector serotype LK03 encoding human ornithine transcarbamylase, EMA/OD/326/16 Modified messenger ribonucleic acid encoding human ornithine transcarbamylase enzyme encapsulated into lipid nanoparticles, EMA/OD/227/15 Adeno-associated viral vector serotype 8 encoding human ornithine transcarbamylase, EMEA/OD/101/07 Heterologous human adult liver derived stem cells

2.2.14. - EMA/OD/065/17

Treatment of Dravet Syndrome

Action: For discussion/adoption

Document(s) tabled:
Draft Summary report

Notes: There have been 3 designations for this condition: See 2.1.1.

2.2.15. - EMA/OD/227/17

Treatment of perinatal asphyxia

Action: For discussion/adoption

Document(s) tabled:
Draft Summary report

Notes: There have been 7 designations for this condition: EMEA/OD/109/09 2-iminobiotin, EMA/OD/133/11 Melatonin, EMA/OD/134/12 Allopurinol sodium, EMA/OD/315/14 Xenon, EMA/OD/004/15 Allopurinol sodium, EMA/OD/042/15 Cannabidiol, EMA/OD/220/16 Pr-D-Cys-Met-Pip-Arg-Leu-Arg-Sar-Cys-Lys-Arg-Pro-Tyr-Tle-Leu-OH

2.2.16. - EMA/OD/226/17

Treatment of Proteus Syndrome

Action: For discussion/adoption

Document(s) tabled:
Draft Summary report

2.2.17. - EMA/OD/231/17

Treatment of diffuse large B-cell lymphoma

Action: For discussion/adoption

Document(s) tabled:
Draft Summary report

Notes: There have been 7 designations for this condition: See 2.1.13.

2.2.18. - EMA/OD/232/17

Treatment of haemophilia B

Action: For discussion/adoption

Document(s) tabled:
Draft Summary report

Notes: There have been 8 designations for this condition: EMEA/OD/117/09 Recombinant fusion protein linking human coagulation factor IX with human albumin, EMA/OD/133/10

Recombinant fusion protein linking human coagulation factor VIIa with human albumin, EMA/OD/090/11 Adeno-associated viral vector containing the human factor IX gene, EMA/OD/041/14 Synthetic double-stranded siRNA oligonucleotide directed against antithrombin mRNA that is covalently linked to a ligand containing three N-acetylgalactosamine residues, EMA/OD/073/14 Recombinant factor VIIa modified with three terminal repeats derived from the β chain of human chorionic gonadotropin, EMA/OD/172/15 Adeno-associated virus viral vector serotype rh10 encoding containing the human factor IX gene, EMA/OD/018/17 Recombinant human factor IX protein modified with three point mutations, EMA/OD/116/17 Concizumab
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

2.2.19. - EMA/OD/230/17

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

Action: For discussion/adoption

Document(s) tabled:
Draft Summary report

Notes: There have been 2 designations for this condition: EMA/OD/018/12 Recombinant adeno-associated viral vector containing human acid alfa-glucosidase-gene, EMA/OD/148/13 recombinant human alpha-glucosidase conjugated with multiple copies of synthetic bismannose-6-phosphate-tetra-mannose glycan
Designation withdrawn: EMEA/OD/001/07 Recombinant adeno-associated viral vector containing human acid alfa-glucosidase-gene

2.2.20. - EMA/OD/224/17

Treatment of Crimean-Congo haemorrhagic fever

Action: For discussion/adoption

Document(s) tabled:
Draft Summary report

2.2.21. - EMA/OD/225/17

Treatment of Lassa fever

Action: For discussion/adoption

Document(s) tabled:
Draft Summary report

2.2.22. - EMA/OD/242/17

Prevention of ricin poisoning

Action: For discussion/adoption

Document(s) tabled:
Draft Summary report

2.2.23. - EMA/OD/228/17

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 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 limpet haemocyanin, EMA/OD/050/11 2-hydroxyoleic acid, EMA/OD/157/11 Adenovirus-associated vector containing human Fas-c gene, EMA/OD/170/12 4-[2-(6-methylpyridin-2-yl)-5,6-dihydro-4H-pyrrolo[1,2-b]pyrazol-3-yl]-quinoline-6-carboxamide monohydrate, EMA/OD/148/12 1,2:5,6-Dianhydrogalactitol, EMA/OD/086/13 Autologous ex vivo expanded leukocytes treated with 5-aza-2'-deoxycytidine, EMA/OD/001/14 Autologous dendritic cells pulsed with RNA from glioma stem cells, EMA/OD/107/13 Allogeneic and autologous haptenised and irradiated cells and cell lysates derived from glioma, EMA/OD/174/13 Autologous dendritic cells pulsed with tumour antigen-derived synthetic peptides (MAGE-1, HER-2, AIM-2, TRP-2, gp-100, and interleukin-13 receptor alpha), EMA/OD/111/14 Recombinant human bone morphogenetic protein 4, EMA/OD/003/14 Paclitaxel-succinate- Arg-Arg-Leu-Ser-Tyr-Ser-Arg-Arg-Arg-Phe, EMA/OD/065/14 Humanised recombinant monoclonal antibody against epidermal growth factor receptor conjugated to maleimidocaproyl monomethylauristatin F, EMA/OD/132/14 Olaptesed pegol, EMA/OD/200/14 5,5'-(4-(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

2.2.24. - EMA/OD/234/17

Treatment of malignant mesothelioma

Action: For discussion/adoption

Document(s) tabled:
Draft Summary report

Notes: There have been 11 designations for this condition: EMEA/OD/003/08 NGR-human Tumour Necrosis Factor, EMA/OD/063/12 Maytansinoid-conjugated human monoclonal antibody against mesothelin, EMA/OD/012/13 N-methyl-4-({4-[({3-methyl(methylsulfonyl)aminopyrazin-2-yl}methyl)amino]-5-(trifluoromethyl)pyrimidin-2-yl}amino)benzamide hydrochloride, EMA/OD/138/13 Autologous dendritic cells pulsed with allogeneic tumour cell lysate, EMA/OD/108/13 Amatuximab, EMA/OD/076/14 Pegylated recombinant arginine deiminase, EMA/OD/180/14 Genetically modified serotype 5/3 adenovirus coding for granulocyte macrophage colony-stimulating factor, EMA/OD/157/15 Live attenuated Listeria monocytogenes delta actA/delta inlB strain expressing human mesothelin, EMA/OD/243/15 Tyr-Met-Phe-Pro-Asn-Ala-Pro-Tyr-Leu, Ser-Gly-Gln-Ala-Tyr-Met-Phe-Pro-Asn-Ala-Pro-Tyr-Leu-Pro-Ser-Cys-Leu-Glu-Ser, Arg-Ser-Asp-Glu-Leu-Val-Arg-His-His-Asn-Met-His-Gln-Arg-Asn-Met-Thr-Lys-Leu and Pro-Gly-Cys-Asn-Lys-Arg-Tyr-Phe-Lys-Leu-Ser-His-Leu-Gln-Met-His-Ser-Arg-Lys-His-Thr-Gly, EMA/OD/101/16 Cisplatin, EMA/OD/250/16 Doxorubicin hydrochloride in a lipid-based pegylated nanoparticle modified with a 31-aminoacid peptide targeting nucleolin
Designations withdrawn: EMEA/OD/022/01 Pemetrexed disodium, EMA/OD/028/10 Vorinostat, EMA/OD/168/14 5-[8-methyl-9-(1-methylethyl)-2-(4-morpholinyl)-9H-purin-6-yl]-2-pyrimidinamine

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/127/17

Therapicon Srl; Treatment of subarachnoid hemorrhage

Action: For adoption, Oral explanation to be held on 13 February 2018 at time 11:00

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 13-15 February 2018 COMP meeting

2.7. Evaluation on-going

Thirteen 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 Niemann-Pick disease, type C

Action: For adoption

3.1.2. -

Treatment of mucopolysaccharidosis type I

Action: For adoption

3.1.3. -

Treatment of glioma

Action: For adoption

3.1.4. -

Treatment of paroxysmal nocturnal haemoglobinuria

Action: For adoption

3.1.5. -

Treatment of primary biliary cholangitis

Action: For adoption

3.1.6. -

Treatment of primary focal segmental glomerulosclerosis

Action: For adoption

3.1.7. -

Treatment of adenosine deaminase-deficient-severe combined immunodeficiency

Action: For adoption

3.1.8. -

Treatment of acute hepatic porphyria

Action: For adoption

3.1.9. -

Treatment of adrenoleukodystrophy

Action: For adoption

3.2. Finalised letters

3.2.1. -

Treatment of acute myeloid leukaemia

Action: For information

3.2.2. -

TKI inhibitor for treatment of gastrointestinal stromal tumors

Action: For information

3.2.3. -

Treatment of cutaneous T-cell lymphoma

Action: For information

3.2.4. -

Treatment of diffuse large B-cell lymphoma

Action: For information

3.2.5. -

Treatment of multiple myeloma

Action: For information

3.2.6. -

Treatment of multiple myeloma

Action: For information

3.3. New requests

3.3.1. -

Treatment of eosinophilic oesophagitis

Action: For information

3.3.2. -

Treatment of gastrointestinal stromal tumours

Action: For information

3.3.3. -

Treatment of pulmonary arterial hypertension

Action: For information

3.3.4. -

Treatment of partial deep dermal and full thickness burns

Action: For information

3.3.5. -

Treatment of amyotrophic lateral sclerosis

Action: For information

3.3.6. -

Treatment of sickle cell disease

Action: For information

3.3.7. -

Treatment of soft tissue sarcoma

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. - gemtuzumab ozogamicin – EMEA/H/C/004204, EMEA/OD/022/00, EU/3/00/005

Pfizer Limited; Treatment of acute myeloid leukaemia (AML)

Action: For discussion

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

4.2.2. - 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

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

Ammtek; Treatment of neonatal diabetes

Action: For information

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

AbbVie Limited; Treatment of chronic lymphocytic leukaemia

CHMP rapporteur: Filip Josephson

Action: For discussion

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

5.2.2. Lynparza - Olaparib – Type II variation – 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; CHMP co-rapporteur: Bart Van der Schueren

Action: For information

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

5.2.3. 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

5.3. 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, Amsterdam, 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 13 February 2018 at 13:00

Document(s) tabled:
PAWG draft agenda for 13 February 2018 meeting
PAWG draft minutes for 16 January 2018 meeting

7.1.3. Non-Clinical Working Group

Proposed meeting time on 14 February 2018 at 08:30

7.1.4. Condition Working Group

Proposed meeting time on 14 February 2018 at 13:00

7.1.5. Prevalence Working Group

Proposed meeting time on 15 February 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 January 2018

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

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

None

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

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/