8 October 2018
EMA/COMP/634073/2018
Inspections, Human Medicines Pharmacovigilance and Committees Division

Committee for Orphan Medicinal Products (COMP)

Draft agenda for the meeting on 9-11 October 2018

Chair: Violeta Stoyanova-Beninska

09 October 2018, 08:30-20:00, room 02-F

10 October 2018, 08:30-20:00, room 02-F

11 October 2018, 08:30-18:00, room 02-F

Health and safety information

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

Disclaimers

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

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

Note on access to documents

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

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1. Introduction

1.1. Welcome and declarations of interest of members and experts

Pre-meeting list of participants and restrictions in relation to declarations of interests applicable to the items of the agenda for the COMP plenary session to be held 9-11 October 2018. See October 2018 COMP minutes (to be published post November 2018 COMP meeting).

1.2. Adoption of agenda

COMP agenda for 9-11 October 2018.

1.3. Adoption of the minutes

COMP minutes for 11-13 September 2018.

2. Applications for orphan medicinal product designation

2.1. For opinion

2.1.1. - EMA/OD/086/18

Treatment of hepatocellular carcinoma

Action: For adoption, Oral explanation to be held on 9 October 2018 at 09:00

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

Notes: There have been 19 designations for this condition: EMEA/OD/015/02 Thymalfasin, EMEA/OD/087/04 Pegylated arginine deiminase, EMEA/OD/048/04 Doxorubicine polyisohexylcyanoacrylate nanoparticles, EMEA/OD/018/05 Nemorubicin hydrochloride, EMEA/OD/070/09 NGR-human tumour necrosis factor, EMEA/OD/076/09 Vaccinia GM-CSF/TK-deactivated virus, EMA/OD/065/10 (S)-10-[(dimethylamino)methyl]-4-ethyl-9-hydroxy-4-O-[alpha-(2", 4", 5", 7"-tetranitro-9"-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/160/14 Diaspirin cross-linked haemoglobin, EMA/OD/087/15 2-(2-phenylvinyl)-4-[4-methylpiperazin-1-yl)]-6-(5-methyl-2H-pyrazol-3-yl-amino)-pyrimidine L(+) tartrate salt, EMA/OD/118/15 2-chloro-N6-(3-iodobenzyl)adenosine-5'-N-methyluronamide, EMA/OD/072/16 Mifamurtide, EMA/OD/052/17 N-{2-[(6-{[(2,6-dichloro-3,5-dimethoxyphenyl)carbamoyl](methyl)amino}pyrimidin-4-yl)amino]-5-(4-ethylpiperazin-1-yl)phenyl}prop-2-enamide, EMA/OD/312/16 Mertansine functionalised gold nanoconjugate, EMA/OD/038/17 Tirapazamine

Designations withdrawn: 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, EMA/OD/115/13 Tivantinib, EMA/OD/287/14 Lenvatinib

2.1.2. - EMA/OD/085/18

Treatment of hepatocellular carcinoma

Action: For adoption, Oral explanation to be held on 9 October 2018 at 09:00

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

Notes: There have been 19 designations for this condition: See 2.1.1.

2.1.3. - EMA/OD/258/17

Treatment of ovarian cancer

Action: For adoption, Oral explanation to be held on 9 October 2018 at 10:00

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

Notes: There have been 29 designations for this condition: EMEA/OD/019/02 Oregovomab, EMEA/OD/080/03 Anti-epithelial cell adhesion molecule/anti-CD3 monoclonal antibody, EMEA/OD/044/03 Trabectedin, EMEA/OD/065/05 Imexon, EMEA/OD/110/07 Humanised monoclonal antibody to the folate receptor alpha, EMEA/OD/006/09 Human MHC non-restricted cytotoxic T-cell line, EMEA/OD/086/09 8-[4-(1-aminocyclobutyl)phenyl]-9-phenyl-1,2,4-triazolo[3,4-f][1,6]naphthyridin-3(2H)-one mono-hydrochloride, EMA/OD/015/10 (3S)-3-{4-[7-(aminocarbonyl)-2H-indazol-2-yl] phenyl} piperidine tosylate monohydrate salt, EMA/OD/021/10 Autologous dendritic cells pulsed with recombinant human-fusion protein (mucin 1 - glutathione S transferase) coupled to oxidised polymannose, EMA/OD/111/10 Veliparib, EMA/OD/054/11 20-pentaerythritol poly (oxy-1,2-ethanediyl)-carboxymethyl-glycinate-7-ethyl-10-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, EMA/OD/246/17 Autologous dendritic cells pulsed with killed ovarian cancer cells and matured by TLR3 ligand ex vivo
Designations withdrawn: EMEA/OD/061/00 Human Milk Fat Globule 1 / Yttrium (90Y) human Milk Fat Globule 1 - S p isothiocyanatobenzyl-diethylenetriaminepentaacetic acid, EMEA/OD/062/01 Epothilone B, EMEA/OD/061/06 Paclitaxel (micellar), EMEA/OD/016/03 Murine anti-idiotypic antibody against OC125 antibody against CA125 antigen, EMEA/OD/063/07 Olaparib, EMEA/OD/071/09 Anti-EphA2 monoclonal antibody conjugated to maleimidocaproyl monomethylauristatin phenylalanine, EMA/OD/014/10 Pyr-His-Trp-Ser-Tyr-D-Lys(doxorubicinylglutarate)-Leu-Arg-Pro-Gly-NH2, acetate salt, EMA/OD/094/11 Vincaleukoblastin-23-oic acid, O4-deacetyl-2-[(2-mercaptoethoxy)carbonyl]hydrazide, disulfide with …, EMA/OD/002/12 1-(4-{4-amino-7-[1-(2-hydroxyethyl)-1H- pyrazol-4-yl] thieno [3,2-c]pyridin-3-yl}phenyl)-3-(3-fluorophenyl)urea, EMA/OD/114/12 Alisertib, EMA/OD/314/14 {2-amino-8-[4-(pyrrolidinylcarbonyl)phenyl]-(3H-benzo[f]azepin-4-yl)}-N,N-dipropylcarboxamide

2.1.4. - EMA/OD/110/18

Treatment of pancreatic cancer

Action: For adoption, Oral explanation to be held on 9 October 2018 at 12:00

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 □-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

2.1.5. - EMA/OD/106/18

Treatment of cystic fibrosis

Action: For adoption, Oral explanation to be held on 9 October 2018 at 14:00

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

Notes: There have been 40 designations for this condition: EMEA/OD/032/00 L-Lysine-N-Acetyl-L-Cysteinate, EMEA/OD/011/03 Recombinant dog gastric lipase, EMEA/OD/038/02 Duramycin, EMEA/OD/039/04 Dexamethasone sodium phosphate encapsulated in human erythrocytes, EMEA/OD/053/04 Alpha-1 antitrypsin (inhalation use), EMEA/OD/062/05 Mannitolum, EMEA/OD/001/06 Heparin sodium, EMEA/OD/037/09 Ciprofloxacin (liposomal), EMEA/OD/092/06 Ciprofloxacin (inhalation use), EMEA/OD/104/06 Alginate oligosaccharide (G-block) fragment, EMEA/OD/041/07 Alpha1-proteinase inhibitor (inhalation use), EMEA/OD/031/08 Avian polyclonal IgY antibody against Pseudomonas aeruginosa, EMEA/OD/010/08 N-(2,4-Di-tert-butyl-5-hydroxyphenyl)-1,4-dihydro-4-oxoquinoline-3-carboxamide, EMEA/OD/009/09 Hypothiocyanite / lactoferrin, EMA/OD/040/10 Nafamostat mesilate, EMA/OD/032/11 Sinapultide, dipalmitoylphosphatidylcholine palmitoyl-oleoyl phosphatidylglycerol, sodium salt and palmitic acid, EMA/OD/037/11 Multilamellar microvesicle comprising phosphatidylcholine, sphingomyelin, phosphatidylethanolamine, phosphatidylserine, 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/203/17 (R)-2-(5-cyano-2-(6-(methoxycarbonyl)-7-methyl-3-oxo-8-(3-(trifluoromethyl)phenyl)-2,3,5,8-tetrahydro-[1,2,4]triazolo[4,3-a]pyrimidine-5-yl)phenyl)-N,N,N-trimethylethanaminium methanesulfonate dehydrate, EMA/OD/303/16 Phosphoinositide 3-kinase gamma peptide, EMA/OD/006/17 Tamoxifen citrate, EMA/OD/085/17 Teicoplanin

Designations withdrawn: EMEA/OD/009/02 Carbamic acid /[[4-[[3-[[4-[1-(4-hydroxyphenyl)-1-methyl-ethyl]phenoxy]methyl]phenyl]methoxy]-phenyl]iminomethyl]-,ethyl ester, EMEA/OD/064/00 8-cyclopentyl-1, 3-dipropylxanthine, EMEA/OD/018/03 Engineered protein inhibitor of human neutrophil elastase, EMEA/OD/075/02 Amiloride hydrochloride dihydrate, EMEA/OD/023/04 Recombinant human bile salt-stimulated lipase, EMEA/OD/107/04 3-[5-(2-fluoro-phenyl)-[1,2,4]oxadiazole-3-yl]-benzoic acid, EMEA/OD/054/05 Heparin sodium (inhalation use), EMEA/OD/072/05 Denufosol tetrasodium, EMEA/OD/118/05 Glutathione, EMEA/OD/024/08 Levofloxacin hemihydrate, EMA/OD/024/10 3-(6-(1-(2,2-difluorobenzo [d] [1,3] dioxol-5-yl)cyclopropanecarboxamido)-3-methylpyridin-2-yl)benzoic acid, EMA/OD/032/14 Lumacaftor/ivacaftor

2.1.6. - EMA/OD/093/18

Treatment of anti-glomerular basement membrane disease

Action: For adoption, Oral explanation to be held on 9 October 2018 at 15:00

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

2.1.7. - EMA/OD/108/18

Treatment of noninsulinoma pancreatogenous hypoglycaemia syndrome

Action: For adoption, Oral explanation to be held on 9 October 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/065/16 Exendin (9-39)

2.1.8. - EMA/OD/128/18

Treatment of graft-versus-host disease

Action: For adoption, Oral explanation to be held on 9 October 2018 at 18:00

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

Notes: There have been 14 designations for this condition: EMEA/OD/038/00 Inolimomab, EMEA/OD/046/05 A mixture of anti-CD3 mAb (SPV-T3a)-ricin A chain fusion protein and anti-CD7 mAb (WT1)-ricin A chain fusion protein, EMEA/OD/009/06 Methoxsalen, EMEA/OD/049/06 Budesonide (oral use), EMEA/OD/068/06 Ex-vivo cultured adult human mesenchymal stem cells, EMA/OD/022/10 Murine monoclonal antibody against CD26, EMA/OD/197/12 Allogeneic bone marrow derived mesenchymal cells expanded ex vivo in synthetic media, EMA/OD/267/14 Human plasma-derived alpha-1 proteinase inhibitor, EMA/OD/017/16 Rimiducid, EMA/OD/110/16 Cannabidiol, EMA/OD/178/16 Ibrutinib, EMA/OD/208/16 Arsenic trioxide, EMA/OD/169/17 Itacitinib, EMA/OD/023/18 Allogeneic bone marrow derived mesenchymal stromal cells, ex-vivo expanded
Designations withdrawn: EMEA/OD/020/00 Thalidomide, EMA/OD/118/16 Recombinant humanised monoclonal antibody against human complement component C5a

2.1.9. - EMA/OD/096/18

Treatment of ATTR amyloidosis

Action: For adoption, Oral explanation to be held on 9 October 2018 at 19:00

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

Notes: There has been 1 designation for this condition: EMA/OD/098/13 Phosphorothioate oligonucleotide targeted to transthyretin
Designation withdrawn: EMA/OD/194/13 Synthetic double-stranded siRNA oligonucleotide directed against transthyretin mRNA and covalently linked to a ligand containing three N-acetylgalactosamine residues

2.1.10. - EMA/OD/116/18

Treatment of glioma

Action: For adoption, Oral explanation to be held on 9 October 2018 at 16:00

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

Notes: There have been 47 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-(trifluromethyl) 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, EMA/OD/185/17 Vocimagene amiretrorepvec, EMA/OD/198/17 Flucytosine, EMA/OD/252/17 H-Arg-Pro-Lys-Pro-Gln-Gln-Phe-2Thi-Gly-Leu-Met(O2)-NH2-DOTA-225-actinium, EMA/OD/036/18 Combination of carboplatin and sodium valproate, EMA/OD/049/18 Autologous glioma tumour cells treated with antisense molecule directed against the insulin-like growth factor type 1 receptor, EMA/OD/060/18 (3R,3aS,9R,9aS,9bS)-3-((dimethylamino)methyl)-9-hydroxy-6,9-dimethyl-3,3a,4,5,7,8,9,9a-octahydroazuleno[4,5-b]furan-2(9bH)-one fumarate

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

2.1.11. - EMA/OD/117/18

Treatment of papillary thyroid cancer

Action: For adoption, Oral explanation to be held on 9 October 2018 at 16:00

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

Notes: There has been 1 designation for this condition: EMA/OD/093/13 Sorafenib tosylate
Designation withdrawn: EMA/OD/173/12 Lenvatinib

2.1.12. - EMA/OD/118/18

Treatment of anaplastic thyroid cancer

Action: For adoption, Oral explanation to be held on 9 October 2018 at 16:00

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

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

2.1.13. - EMA/OD/111/18

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

Action: For adoption, Oral explanation to be held on 10 October 2018 at 11:00

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

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

2.1.14. - EMA/OD/122/18

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

Action: For adoption, Oral explanation to be held on 10 October 2018 at 14:00

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

2.1.15. - EMA/OD/129/18

Prevention of graft versus host disease

Action: For adoption, Oral explanation to be held on 10 October 2018 at 15:00

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

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

2.1.16. - EMA/OD/084/18

Prevention of bronchopulmonary dysplasia

Action: For adoption, Oral explanation to be held on 10 October 2018 at 16:00

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

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

2.1.17. - EMA/OD/126/18

Treatment of neuroblastoma

Action: For adoption, Oral explanation to be held on 10 October 2018 at 17:00

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

Notes: There have been 10 designations for this condition: EMEA/OD/096/07 Iodine (131I) iobenguane, EMEA/OD/093/09 16-base single-stranded PNA oligonucleotide linked to a 7-aminoacid peptide, EMA/OD/126/10 Eflornithine, EMA/OD/002/11 Chimeric monoclonal antibody against GD2, EMA/OD/020/12 16 base single stranded peptide nucleic acid oligonucleotide - 7 aminoacids peptide, EMA/OD/112/12 Chimeric monoclonal antibody against GD2, EMA/OD/199/14 Chimeric monoclonal antibody specific to O-acetyl-GD2 antigen, EMA/OD/326/14 Sodium 2-hydroxylinoleate, EMA/OD/136/15 N-[5-(3,5-difluorobenzyl)-1H-indazol-3-yl]-4-(4 methylpiperazin-1-yl)-2-(tetrahydro-2H-pyran-4-ylamino)benzamide, EMA/OD/271/16 Iodine (131I) murine IgG1 monoclonal antibody against CD276
Designation withdrawn: EMEA/OD/013/09 Murine monoclonal antibody to GD2

2.1.18. - EMA/OD/124/18

Treatment of primary mediastinal large-B-cell lymphoma

Action: For adoption, Oral explanation to be held on 10 October 2018 at 18:00

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

2.1.19. - EMA/OD/115/18

Treatment of Short Bowel Syndrome

Action: For adoption, Oral explanation to be held on 10 October 2018 at 19:00

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

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

2.1.20. - EMA/OD/121/18

Treatment of follicular lymphoma

Action: For information

Document(s) tabled:
Withdrawal request of 21 September 2018

Notes: There have been 11 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, EMA/OD/260/17 Autologous CD4+ and CD8+ T cells expressing a CD19-specific chimeric antigen receptor, EMA/OD/222/17 Tazemetostat
Designations withdrawn: EMEA/OD/061/02 Iodine (131I) tositumomab, EMEA/OD/079/02 Tositumomab, EMA/OD/053/13 Idelalisib

2.1.21. - EMA/OD/097/18

Treatment of alcohol-dependence

Action: For information

Document(s) tabled:
Withdrawal request of 21 September 2018

2.1.22. - EMA/OD/119/18

Treatment of large hemispheric infarction

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

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

2.1.23. - EMA/OD/103/18

Treatment of Focal Segmental Glomerulosclerosis

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

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

Notes: There has been 1 designation for this condition: EMA/OD/115/15 4'-[(2-Butyl-4-oxo-1,3-diazaspiro[4.4]non-1-en-3-yl)methyl]-N-(4,5-dimethyl-3-isoxazolyl)-2'-(ethoxymethyl)-[1,1'-biphenyl]-2-sulfonamide
Designation withdrawn: EMA/OD/146/10 Fresolimumab

2.1.24. - EMA/OD/040/18

Treatment of Kabuki syndrome

Action: For adoption

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

2.1.25. - EMA/OD/130/18

Treatment of severe combined immunodeficiency

Action: For information

Document(s) tabled:
Withdrawal request of 20 September 2018

2.2. For discussion / preparation for an opinion

2.2.1. - EMA/OD/150/18

Treatment of pseudomyxoma peritonei

Action: For discussion/adoption

Document(s) tabled:
Draft Summary report

2.2.2. - EMA/OD/140/18

Treatment of congenital adrenal hyperplasia

Action: For discussion/adoption

Document(s) tabled:
Draft Summary report

Notes: There have been 3 designations for this condition: EMEA/OD/020/05 Hydrocortisone (modiefied release tablet), EMA/OD/164/17 Pyrazolo[1,5-a]pyrimidine, 3-[4-chloro-2-(4-morpholinyl)-5-thiazolyl]-7-(1-ethylpropyl)-2,5-dimethyl-pyrazolo[1,3-a]pyrimidine,

EMA/OD/179/17 N-[2,6-bis(1-methylethyl)phenyl]-N'-[[1-[4-(dimethylamino)phenyl]cyclopentyl]methyl]urea, hydrochloride salt
Designation withdrawn: EMA/OD/063/15 Verucerfont

2.2.3. - EMA/OD/141/18

Treatment of Fanconi anaemia

Action: For discussion/adoption

Document(s) tabled:
Draft Summary report

2.2.4. - EMA/OD/123/18

Treatment of graft-versus-host disease

Action: For discussion/adoption

Document(s) tabled:
Draft Summary report

Notes: There have been 14 designations for this condition: See 2.1.8.

2.2.5. - EMA/OD/144/18

Treatment of pseudomyxoma peritonei

Action: For discussion/adoption

Document(s) tabled:
Draft Summary report

2.2.6. - EMA/OD/131/18

Treatment of epidermolysis bullosa

Action: For discussion/adoption

Document(s) tabled:
Draft Summary report

Notes: There have been 15 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, EMA/OD/244/17 Genetically modified replication- incompetent herpes simplex virus-1 expressesing collagen VII
Designation withdrawn: EMA/OD/172/10 Human dermal fibroblasts cultured on a bioresorbable polyglactin mesh

2.2.7. - EMA/OD/135/18

Treatment of Hereditary Hemorrhagic Telangiectasia

Action: For discussion/adoption

Document(s) tabled:
Draft Summary report

2.2.8. - EMA/OD/134/18

Treatment of Duchenne Muscular Dystrophy

Action: For discussion/adoption

Document(s) tabled:
Draft Summary report

Notes: There have been 31 designations for this condition: EMEA/OD/106/04 3-[5-(2-fluoro-phenyl)-[1,2,4]oxadiazole-3-yl]-benzoic acid, EMEA/OD/026/05 Adeno-associated viral vector containing a modified U7 snRNA gene, EMEA/OD/077/06 Idebenone, EMEA/OD/065/08 5-(ethylsulfonyl)-2-(naphthalen-2-yl)benzo[d]oxazole, EMEA/OD/049/08 RNA, [P-deoxy-P-(dimethylamino)] (2',3'-dideoxy-2',3'-imino-2',3'-seco) (2'a→5') (C-m5U-C-C-A-A-C-A-m5U-C-A-A-G-G-A-A-G-A-m5U-G-G-C-A-m5U-m5U-m5U-C-m5U-A-G), P-[4-[[2-[2-(2-ydroxyethoxy)ethoxy]ethoxy]carbonyl]-1-piperazinyl] N,N dimethylaminophosphonamidate, EMEA/OD/081/08 Exon 44 specific phosphorothioate oligonucleotide, EMEA/OD/082/08 Exon 51 specific phosphorothioate oligonucleotide, EMEA/OD/044/09 Adeno-associated viral vector containing modified U1 snRNA, EMEA/OD/083/09 RNA, [P-deoxy-P-(dimethylamino)] (2',3'-dideoxy-2',3'-imino-2',3'-seco) (2'a→5') (C-m5U-m5U-A-C-A-G-G-C-m5U-C-C-A-A-m5U-A-G-m5U-G-G-m5U-C-A-G-m5U), 5' [P-[4-[[2-[2-(2-hydroxyethoxy)ethoxy]ethoxy]carbonyl]-1-piperazinyl]-N,N-dimethylaminophosphonamidate], 3'-[2'a-[N2-acetyl-L-arginyl-6-aminohexanoyl-L-arginyl-L-arginyl-β-alanyl-L-arginyl-L-arginyl-6-aminohexanoyl-L-arginyl-L-arginyl-β-alanyl-L-arginyl-6-aminohexanoyl-β-alanyl], octahydrochloride, EMEA/OD/049/08 RNA, [P-deoxy-P-(dimethylamino)] (2',3'-dideoxy-2',3'-imino-2',3'-seco) (2'a→5') (C-m5U-C-C-A-A-C-A-m5U-C-A-A-G-G-A-A-G-A-m5U-G-G-C-A-m5U-m5U-m5U-C-m5U-A-, EMA/OD/142/11 Exon 45 specific phosphorothioate oligonucleotide, EMA/OD/143/11 Exon 53 specific phosphorothioate oligonucleotide, EMA/OD/162/11 Halofuginone hydrobromide, EMA/OD/028/12 Givinostat, EMA/OD/121/12 Exon 52 specific phosphorothioate oligonucleotide, EMA/OD/122/12 Exon 55 specific phosphorothioate oligonucleotide, EMA/OD/164/12 Humanised monoclonal antibody against myostatin, EMA/OD/183/12 R,S-O-(3-piperidino-2-hydroxy-1-propyl)-nicotinic acid amidoxime dihydrochloride,

EMA/OD/162/13 Asp-Arg-Val-Tyr-Ile-His-Pro, EMA/OD/049/14 17α,21-dihydroxy-16α-methyl-pregna-1,4,9(11)-triene-3,20-dione, EMA/OD/166/14 Adeno-associated viral vector serotype 8 containing the human MD1 gene, EMA/OD/307/14 Rimeporide, EMA/OD/041/15 Allogeneic human adult stem cells, isolated from skeletal muscle and expanded ex vivo, EMA/OD/109/15 N-(2-((4Z,7Z,10Z,13Z,16Z,19Z)-docosa-4,7,10,13,16,19-hexaenamido)ethyl)-2-hydroxybenzamide, EMA/OD/161/16 Recombinant adeno-associated viral vector encoding a human micro-dystrophin gene under the control of a muscle specific promoter, EMA/OD/096/16 Adeno-associated viral vector serotype 9 containing the human mini-dystrophin gene, EMA/OD/133/17 Tamoxifen citrate, EMA/OD/154/17 Metformin and L-citrulline, EMA/OD/032/18 Synthetic antisense oligonucleotide directed against human dystrophin pre-mRNA, EMA/OD/020/18 20-hydroxyecdysone, EMA/OD/028/18 Ex-vivo fused autologous human bone marrow-derived mesenchymal stem cell with allogenic human myoblast, EMA/OD/043/18 Tetracosactide
Designations withdrawn: EMEA/OD/096/05 2'-O-methyl-phosphorothioate oligonucleotide, EMEA/OD/025/06 2-(4-(diethylamino) phenyl)-6-methyl-2H-benzo[d][1,2,3] triazol-5-amine, EMA/OD/085/10 Recombinant fusion protein consisting of the extracellular portion of human activin receptor IIB linked to the human IgG1 Fc domain, EMA/OD/090/13 Naproxcinod

2.2.9. - EMA/OD/133/18

Treatment of Duchenne muscular dystrophy

Action: For discussion/adoption

Document(s) tabled:
Draft Summary report

Notes: There have been 31 designations for this condition: See 2.2.8.

2.2.10. - EMA/OD/145/18

Treatment of glioma

Action: For discussion/adoption

Document(s) tabled:
Draft Summary report

Notes: There have been 47 designations for this condition: See 2.1.10.

2.2.11. - EMA/OD/136/18

Treatment of spinal muscular atrophy

Action: For discussion/adoption

Document(s) tabled:
Draft Summary report

Notes: There have been 2 designations for this condition: EMA/OD/028/15 Adeno-associated viral vector serotype 9 containing the human SMN gene, EMA/OD/065/18 Adeno-associated viral vector serotype hu68 containing the human SMN1 gene

2.2.12. - EMA/OD/109/18

Treatment of beta -thalassemia intermedia and major

Action: For discussion/adoption

Document(s) tabled:
Draft Summary report

Notes: There have been 6 designations for this condition: EMA/OD/112/17 Bitopertin, EMA/OD/142/15 Sirolimus, EMA/OD/047/14 ACE-536, EMEA/OD/111/08 Thalagen, EMEA/OD/098/08 2,2-dimethylbutyric acid, sodium salt, EMA/OD/099/18 1-(3-methylbutanoyl)-L-aspartyl-L-threonyl-L-histidyl-L-phenylalanyl-L-prolyl-(L-cystinyl-L-isoleucyl-[(N6-(S)-4-carboxy-4-palmitamidobutanoyl)-L-lysinyl]-L-phenylalanyl-L-glutamyl-L-prolyl-L-arginyl-L-serinyl-L-lysinyl-L-glycinyl-L-cystinyl)-L-lysinamide, disulfide, acetate

2.2.13. - EMA/OD/146/18

Treatment of Multiple System Atrophy

Action: For discussion/adoption

Document(s) tabled:
Draft Summary report

Notes: There have been 1 designation for this condition: EMA/OD/193/14 1-(2-isopropoxyethyl)-2-thioxo-1,2,3,5-tetrahydro-pyrrolo[3,2-d] pyrimidin-4-one

2.2.14. - EMA/OD/139/18

Treatment of cystic fibrosis

Action: For discussion/adoption

Document(s) tabled:
Draft Summary report

Notes: There have been 40 designations for this condition: See 2.1.5.

2.2.15. - EMA/OD/137/18

Treatment of cystic fibrosis

Action: For discussion/adoption

Document(s) tabled:
Draft Summary report

Notes: There have been 40 designations for this condition: See 2.1.5.

2.2.16. - EMA/OD/112/18

Treatment of haemophilia B

Action: For discussion/adoption

Document(s) tabled:
Draft Summary report

Notes: There have been 9 designations for this condition: EMEA/OD/117/09 Recombinant fusion protein linking human coagulation factor IX with human albumin, EMA/OD/133/10 Recombinant fusion protein linking human coagulation factor VIIa with human albumin, EMA/OD/090/11 Adeno-associated viral vector containing the human factor IX gene, EMA/OD/041/14 Synthetic double-stranded siRNA oligonucleotide directed against antithrombin mRNA that is covalently linked to a ligand containing three N-acetylgalactosamine residues, EMA/OD/073/14 Recombinant factor VIIa modified with three terminal repeats derived from the β chain of human chorionic gonadotropin, EMA/OD/172/15 Adeno-associated virus viral vector serotype rh10 encoding containing the human factor IX gene, EMA/OD/018/17 Recombinant human factor IX protein modified with three point mutations, EMA/OD/116/17 Concizumab, EMA/OD/232/17 Recombinant adeno-associated viral vector containing a codon-optimized Padua derivative of human coagulation factor IX cDNA
Designations withdrawn: EMEA/OD/008/08 Pegylated recombinant factor VIIa, EMEA/OD/005/09 Pegylated recombinant human factor IX, EMEA/OD/062/09 Sequence modified human recombinant factor VIIa, EMA/OD/070/12 vatreptacog alfa (activated), EMA/OD/003/15 Adeno-associated viral vector containing the human factor IX gene

2.2.17. - EMA/OD/143/18

Treatment of leptin receptor (LEPR) deficiency

Action: For discussion/adoption

Document(s) tabled:
Draft Summary report

2.2.18. - EMA/OD/142/18

Treatment of biliary tract cancer

Action: For discussion/adoption

Document(s) tabled:
Draft Summary report

Notes: There have been 6 designations for this condition: EMA/OD/199/13 (5R,5aR,8aR,9S)-9-[[4,6-O-[(R)-Ethylidene]-□-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], EMA/OD/172/17 Ivosidenib, EMA/OD/038/18 Pemigatinib

2.2.19. - EMA/OD/148/18

Treatment of autosomal dominant polycystic kidney disease (ADPKD)

Action: For discussion/adoption

Document(s) tabled:
Draft Summary report

2.3. Revision of the COMP opinions

None

2.4. Amendment of existing orphan designations

None

2.5. Appeal

None

2.6. Nominations

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

Action: For adoption

Document(s) tabled:
OMPD applications - appointment of coord. at the 09-11 October 2018 COMP meeting

2.7. Evaluation on-going

Twenty seven 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 naevoid basal-cell carcinoma syndrome (Gorlin syndrome)

Action: For adoption

3.1.2. -

Treatment of ovarian cancer

Action: For adoption

3.1.3. -

Treatment of spinal cord injury

Action: For adoption

3.2. Finalised letters

3.2.1. -

Treatment of glioma

Action: For information

3.2.2. -

Treatment of glioma

Action: For information

3.2.3. -

Treatment of glioma

Action: For information

3.2.4. -

Prevention of graft rejection following solid organ transplantation

Action: For information

3.2.5. -

Treatment of transthyretin-mediated amyloidosis

Action: For information

3.3. New requests

3.3.1. -

Treatment of glioma

Action: For information

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

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

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

Bayer AG; Treatment of haemophilia A

Action: For adoption, Oral explanation to be held on 9 October 2018 at time 11:00

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

Notes: Status of the procedure at the CHMP: CHMP positive opinion adopted in September 2018.

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

Kyowa Kirin Limited; Treatment of cutaneous T-cell lymphoma

Action: For adoption, Oral explanation to be held on 11 October 2018 at time 09:00

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

Notes: Status of the procedure at the CHMP: CHMP positive opinion adopted in September 2018.

4.1.3. Luxturna - voretigene neparvovec - EMEA/H/C/004451

Spark Therapeutics Ireland Ltd

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

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

Action: For adoption

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

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

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

Shire Pharmaceuticals Ireland Limited; Treatment of hereditary angioedema

Action: For adoption, Oral explanation to be held on 10 October 2018 at time 12:00

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

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

LUPIN (EUROPE) LIMITED; Treatment of myotonic disorders

Action: For adoption

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

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

5.1. After adoption of CHMP opinion

5.1.1. 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 adoption, Oral explanation to be held on 11 October 2018 at time 10:00

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

Notes: Status of the procedure at the CHMP: CHMP adopted positive opinion in September 2018.

5.2. Prior to adoption of CHMP opinion

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

Celgene Europe Limited; Treatment of multiple myeloma

CHMP rapporteur: Robert James Hemmings

Action: For discussion

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

5.2.2. Translarna - Ataluren - Type II variation – EMEA/H/C/002720/II/0047, EMEA/OD/106/04, EU/3/05/278

PTC Therapeutics International Limited; Treatment of duchenne muscular dystrophy

CHMP rapporteur: Johann Lodewijk Hillege

Action: For discussion

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

5.2.3. Opsumit - Macitentan - Type II variation – EMEA/H/C/002697/II/0029, EMA/OD/023/11, EU/3/11/909

Actelion Registration Limited; Treatment of pulmonary arterial hypertension

CHMP rapporteur: Concepcion Prieto Yerro

Action: For discussion

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

5.3. Appeal

None

5.4. On-going procedures

Action: For information

Document(s) tabled:
Review of orphan designation for OMP for MA extension - On-going procedures

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

None

7. Organisational, regulatory and methodological matters

7.1. Mandate and organisation of the COMP

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

Action: For information

Documents tabled:
Draft Agenda_SRLM SAWP-COMP_Vienna 2018

7.1.2. Protocol Assistance Working Group (PAWG)

Proposed meeting time on 9 October 2018 at 13:00

Document tabled:
PAWG draft agenda for 9 October 2018 meeting

7.1.3. Election of COMP Vice-Chairperson

Action: For adoption

Documents tabled:
COMP Rules of Procedure EMEA/COMP/8212/00 Rev. 4
Procedure for the election of the COMP vice-chairperson

7.1.4. Prevalence Working Group

Proposed meeting time on 10 October 2018 at 13:00

Document tabled:
Draft agenda for 10 October 2018 meeting

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 September 2018

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:

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

Action: For information

Document(s) tabled:

7.4. Cooperation within the EU regulatory network

7.4.1. European Commission

None

7.5. Cooperation with International Regulators

7.5.1. Food and Drug Administration (FDA)

Action: For information

Notes: Monthly teleconference

7.5.2. Japanese Pharmaceuticals and Medical Devices Agency (PMDA)

Action: For information

Notes: Ad hoc basis meeting

7.5.3. The Therapeutic Goods Administration (TGA), Australia

Action: For information

Notes: Ad hoc basis meeting

7.5.4. Health Canada

Action: For information

Notes: Ad hoc basis meeting

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

None

7.7. COMP work plan

Action: For discussion

Document(s) tabled:
Draft COMP Work Plan 2019

7.8. Planning and reporting

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

Action: For information

7.8.2. Overview of orphan marketing authorisations/applications

Action: For information

8. Any other business

8.1. IRIS: a new way of supporting COMP procedures with a CRM (Customer Relationship Management software)

Action: For discussion

Document tabled:
Presentation

8.2. Concepts of significant benefit (follow-up to COMP Work Plan 2017)

Action: For discussion

8.3.

Action: For discussion

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/