16 July 2018
EMA/COMP/425925/2018
Inspections, Human Medicines Pharmacovigilance and Committees Division

Committee for Orphan Medicinal Products (COMP)

Draft agenda for the meeting on 17-19 July 2018

Chair: Bruno Sepodes

17 July 2018, 08:30-20:30, room 2F

18 July 2018, 08:00-20:30, room 2F

19 July 2018, 08:00-15:00, 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).

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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 17-19 July 2018. See July 2018 COMP minutes (to be published post September 2018 COMP meeting).

1.2. Adoption of agenda

COMP agenda for 17-19 July 2018.

1.3. Adoption of the minutes

COMP minutes for 19-21 June 2018.

2. Applications for orphan medicinal product designation

2.1. For opinion

2.1.1. - EMA/OD/058/18

Treatment of progressive supranuclear palsy

Action: For adoption, Oral explanation to be held on 17 July 2018 at 09:00

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

Notes: There have been 5 designations for this condition: EMA/OD/076/10 Methylthioninium, EMA/OD/261/14 N-(3-(4-(3-(diisobutylamino)propyl)piperazin-1-yl)propyl)-1H-benzo[d]imidazol-2-amine disulphate salt, EMA/OD/044/15 Humanised IgG4 monoclonal antibody against extracellular tau, EMA/OD/193/15 Tolfenamic acid, EMA/OD/239/15 Humanised recombinant IgG4 anti-human tau antibody
Designations withdrawn: EMEA/OD/074/09 4-benzyl-2-naphtalen-1-yl-1,2,4-thiadiazolidine-3,5-dione, EMEA/OD/129/09 Davunetide

2.1.2. - EMA/OD/046/18

Treatment of eosinophilic oesophagitis

Action: For information

Document(s) tabled:
Withdrawal request of 9 July 2018
Notes: There have been 4 designations for this condition: EMA/OD/078/13 Budesonide, EMA/OD/118/13 Human monoclonal antibody against human interleukin 13, EMA/OD/004/16 Humanised monoclonal antibody targeting interleukin-15, EMA/OD/230/16 Fluticasone propionate

2.1.3. - EMA/OD/051/18

Treatment of acute myeloid leukaemia

Action: For adoption, Oral explanation to be held on 17 July 2018 at 18:00

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

Notes: There have been 54 designations for this condition: EMEA/OD/022/00 Gemtuzumab ozogamicin, EMEA/OD/028/04 Midostaurin, EMEA/OD/056/06 Antisense oligonucleotide 5'-d[P-Thio] (CCCTG CTCCC CCCTG GCTCC)-3' (see comments box for cenersen sodium), EMEA/OD/098/04 Tipifarnib, EMEA/OD/094/04 Histamine dihydrochloride, EMEA/OD/066/05 1,2-bis(methylsulphonyl)-1-(2-chloroethyl)-2-[(methylamino)carbonyl]hydrazine, EMEA/OD/100/05 zosuquidar trihydrochloride, EMEA/OD/004/06 Decitabine, EMEA/OD/049/07 5'-O-(trans-9"-octadecenoyl)-1-ß-D-arabinofuranosyl cytosine, EMEA/OD/087/07 Recombinant human histone H1.3 and recombinant human N-bis-met-histone H1.3, EMEA/OD/085/07 Azacitidine, EMEA/OD/099/07 N-(2-amino-phenyl)-4-[(4-pyridin-3-yl-pyrimidin-2-ylamino)-methyl] benzamide, EMEA/OD/118/07 Ribonucleotide reductase R2 specific phosphorothioate oligonucleotide, EMEA/OD/015/08 Sapacitabine, EMEA/OD/048/08 Daunorubicin (liposomal), EMEA/OD/105/08 N-(5-tert-Butylisoxazol-3-yl)-N'-{4-[7-(2-(morpholin-4-yl)ethoxy) imidazo[2,1-b][1,3]benzothiazol-2-yl]phenyl}urea dihydrochloride salt, EMEA/OD/028/09 Tosedostat, EMEA/OD/091/09 1-Cyclopropyl-3-[3-(5-morpholin-4-ylmethyl-1H-benzoimidazol-2-yl)-1H-pyrazol-4-yl]-urea, EMEA/OD/147/09 2-methoxymethyl-2-hydroxymethyl-1-azabicyclo[2,2,2]octan-3-one, EMA/OD/044/10 Allogeneic T cells encoding an exogenous TK gene, EMA/OD/094/10 N-[(2S)-2,3-dihydroxypropyl]-3-[(2-fluoro-4-iodophenyl) amino] isonicotinamide hydrochloride, EMA/OD/101/11 Allogeneic human dendritic cells derived from a CD34+ progenitor cell line, EMA/OD/070/11 Liposomal combination of cytarabine and daunorubicin, EMA/OD/158/11 Vosaroxin, EMA/OD/167/12 L-asparaginase encapsulated in erythrocytes, EMA/OD/064/13 trans-N1-((1R,2S)-2-phenylcyclopropyl)cyclohexane-1,4-diamine bis-hydrochloride, EMA/OD/141/13 (2R,3R,4S,5R)-2-(6-amino-9H-purin-9-yl)-5-((((1r,3S)-3-(2-(5-(tert-butyl)-1Hbenzo[d]imidazol-2-yl)ethyl)cyclobutyl)(isopropyl) amino)methyl) tetrahydrofuran-3,4-diol, EMA/OD/181/13 Volasertib, EMA/OD/100/14 4-{[(2R,3S,4R,5S)-4-(4-Chloro-2-fluoro-phenyl)-3-(3-chloro-2-fluoro-phenyl)-4-cyano-5-(2,2-dimethyl-propyl)-pyrrolidine-2-carbonyl]-amino}-3-methoxy-benzoic acid, EMA/OD/061/14 (Z)-3-(3-(3,5-bis(trifluoromethyl)phenyl)-1H-1,2,4-triazol-1-yl)-N'-(pyrazin-2-yl)acrylohydrazide, EMA/OD/103/14 Donor T lymphocytes depleted ex vivo of host alloreactive T cells using photodynamic treatment, EMA/OD/175/14 Allogeneic ex vivo-generated natural killer cells from CD34+ umbilical cord blood progenitor cells, EMA/OD/240/14 Alvocidib, EMA/OD/258/14 Ulocuplumab, EMA/OD/045/15 inecalcitol, EMA/OD/037/15 2-((3-((4-((3-aminopropyl)amino)butyl)amino)propyl)amino)-N-((5S,5aS,8aR,9R)-9-(4-hydroxy-3,5-dimethoxyphenyl)-8-oxo-5,5a,6,8,8a,9-hexahydrofuro[3',4':6,7]naphtho[2,3-d][1,3]dioxol-5-yl)acetamide, tetrahydrochloride, EMA/OD/089/15 CD33-directed antibody-drug conjugate consisting of an antibody conjugated to a DNA cross-linking pyrrolobenzodiazepine dimer drug, EMA/OD/112/15 Recombinant human interleukin-3 truncated diphtheria toxin fusion protein, EMA/OD/145/15 Humanised monoclonal antibody of the IgG4 kappa isotype targeting CD47, EMA/OD/165/15 Sodium (2R,3S,5R)-5-(4-amino-2-oxo-1,3,5-triazin-1(2H)-yl)-2-(hydroxymethyl)tetrahydrofuran-3-yl ((2R,3S,5R)-5-(2-amino-6-oxo-1H-purin-9(6H)-yl)-3-hydroxytetrahydrofuran-2-yl)methyl phosphate, EMA/OD/144/15 Combretastatin A1-diphosphate, EMA/OD/180/15 Arsenic trioxide,

EMA/OD/205/15 Venetoclax, EMA/OD/233/15 Tyr-Met-Phe-Pro-Asn-Ala-Pro-Tyr-Leu, Ser-Gly-Gln-Ala-Tyr-Met-Phe-Pro-Asn-Ala-Pro-Tyr-Leu-Pro-Ser-Cys-Leu-Glu-Ser, Arg-Ser-Asp-Glu-Leu-Val-Arg-His-His-Asn-Met-His-Gln-Arg-Asn-Met-Thr-Lys-Leu and Pro-Gly-Cys-Asn-Lys-Arg-Tyr-Phe-Lys-Leu-Ser-His-Leu-Gln-Met-His-Ser-Arg-Lys-His-Thr-Gly, EMA/OD/253/15 2-methyl-1-[(4-[6-(trifluoromethyl)pyridin-2-yl]-6-{[2-(trifluoromethyl)pyridin-4-yl]amino}-1,3,5-triazin-2-yl)amino]propan-2-ol methanesulfonate, EMA/OD/155/16 P-ethoxy growth factor receptor-bound protein 2 (Grb2) antisense oligonucleotide, EMA/OD/197/16 Ivosidenib, EMA/OD/319/16 225Ac-lintuzumab, EMA/OD/106/17 Glasdegib maleate, EMA/OD/010/17 Sodium (1R, 3R, 4R, 5S)-3-({2-N-acetylamino-2-deoxy-3-O-[(1S)-1-carboxylato-2-cyclohexylethyl]-β-D-galactopyranosyl}oxy)-4-({6-deoxy-α-L-galactopyranosyl}oxy)-5-ethyl-cyclohexan-1-yl-(38-oxo-2,5,8,11,14,17,20,23,26,29,32,35-dodecaoxa-39-azahentetracontan-41-yl) carboxamide, EMA/OD/040/17 Entospletinib, EMA/OD/101/17 Pracinostat, EMA/OD/175/17 Gilteritinib, EMA/OD/193/17 6-{[(1R,2S)-2-aminocyclohexyl]amino}-7-fluoro-4-(1-methyl-1H-pyrazol-4-yl)-1,2-dihydro-3H-pyrrolo[3,4-c]pyridin-3-one monocitrate

Designations withdrawn: EMEA/OD/065/02 2-chloro-9-[2-deoxy-2-fluoro-ß-D-arabinofuranosyl]adenine, EMEA/OD/051/04 Homoharringtonine, EMEA/OD/059/04 Val-Leu-Gln-Glu-Leu-Asn-Val-Thr-Val (Pr1 nanopeptide, sequence 169-177, of proteinase 3), EMEA/OD/045/05 Troxacitabine, EMEA/OD/018/06 Human monoclonal antibody against inhibitory killer cell lg-like receptors (1-7 F9), EMEA/OD/020/06 Lestaurtinib, EMEA/OD/024/07 Arsenic trioxide, EMEA/OD/069/07 Amonafide L-malate, EMEA/OD/060/08 2-[[3-({4-[(5-{2-[(3-Fluorophenyl)amino]-2-oxoethyl}-1H-pyrazol-3-yl)amino]-quinazolin-7-yl}oxy)propyl](ethyl)amino]ethyl dihydrogen phosphate trihydrate, EMEA/OD/118/08 Lintuzumab, EMEA/OD/090/08 Allogeneic ex vivo expanded umbilical cord blood cells, EMEA/OD/016/09 26 base single stranded phosphodiester DNA oligonucleotide, EMEA/OD/132/09 (1S, 2S, 3R, 4R)-3-(5-Fluoro-2-(3-methyl-4-(4-methylpiperazin-1-yl)-phenylamino)-pyrimidin-4-ylamino)-bicyclo[2.2.1]hept-5-ene-2-carboxamide benzoate), EMA/OD/023/10 1-[2-(Benzo[1,2,5]thiadiazol-5-ylamino)-6-(2,6-dichloro-phenyl)-pyrido[2,3-d]pyrimidin-7-yl]-3-tert-butyl-urea, EMA/OD/161/10 Allogeneic bone marrow stem cells treated ex vivo with 16,16-dimethyl prostaglandin E2, EMA/OD/156/10 Allogeneic umbilical cord blood cells treated ex vivo with 16,16-dimethyl prostaglandin E2, EMA/OD/067/11 1-(4-{4-amino-7-[1-(2-hydroxyethyl)-1H- pyrazol-4-yl]thieno[3,2-c]pyridin-3-yl}phenyl)-3-(3-fluorophenyl)urea, EMA/OD/105/12 Liposomal daunorubicin, EMA/OD/188/14 Allogeneic, umbilical cord blood-derived, ex vivo-expanded, haematopoietic CD133+ cells / allogeneic, umbilical cord blood-derived, non-expanded, haematopoietic CD133- cells

2.1.4. - EMA/OD/072/18

Treatment of follicular lymphoma

Action: For adoption, Oral explanation to be held on 17 July 2018 at 19:00

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

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

2.1.5. - EMA/OD/065/18

Treatment of spinal muscular atrophy

Action: For adoption, Oral explanation to be held on 18 July 2018 at 09:00

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

Notes: There has been 1 designation for this condition: EMA/OD/028/15 Adeno-associated viral vector serotype 9 containing the human SMN gene

2.1.6. - EMA/OD/041/18

Treatment of growth hormone deficiency

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

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

Notes: There have been 3 designations for this condition: EMA/OD/133/12 Recombinant modified human growth hormone, EMA/OD/074/13 Recombinant human growth hormone modified by fusion with two hydrophilic polypeptide chains, EMA/OD/013/17 Ibutamoren mesilate

2.1.7. - EMA/OD/039/18

Treatment of idiopathic pulmonary fibrosis

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

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

Notes: There have been 11 designations for this condition: EMEA/OD/052/04 Pirfenidone, EMEA/OD/054/07 Interferon gamma, EMEA/OD/104/09 Macitentan, EMA/OD/079/10 2-(2-chlorophenyl)-4-[3-(dimethylamino)phenyl]-5-methyl-1H-pyrazolo[4,3-C]pyridine-3,6(2H,5H)-dione, EMA/OD/048/12 Recombinant human pentraxin-2, EMA/OD/186/12 nintedanib, EMA/OD/051/14 Humanised anti-alpha v beta 6 monoclonal antibody, EMA/OD/130/14 1-(6-benzothiazolylsulfonyl)-5-chloro-1H-indole-2-butanoic acid, EMA/OD/072/15 3-pentylbenzeneacetic acid sodium salt, EMA/OD/046/16 3-[4-(1H-imidazol-1-ylmethyl)phenyl]-5-(2-methylpropyl)thiophene-2-[(N-butyloxylcarbamate)-sulphonamide] sodium salt, EMA/OD/088/16 2-((2-ethyl-6-(4-(2-(3-hydroxyazetidin-1-yl)-

2-oxoethyl)-piperazin-1-yl)-8-methylimidazo[1,2-alpha]pyridin-3-yl)-(methyl)amino)-4-(4-fluorophenyl)-thiazole-5-carbonitrile

Designations withdrawn: EMEA/OD/002/05 Interferon gamma, EMEA/OD/033/04 Heparin-Sodium, EMEA/OD/075/04 Acetylcysteine, EMEA/OD/105/07 Recombinant human monoclonal antibody against transforming growth factor beta-1, 2 and 3, EMEA/OD/027/08 Bosentan, EMA/OD/029/10 Ambrisentan, EMA/OD/091/11 4-[[9-[(3S)-tetrahydro-3-furanyl]-8-[(2,4,6-trifluorophenyl)amino]-9H-purin-2-yl]amino]-trans-cyclohexanol, EMA/OD/111/12 Tralokinumab

2.1.8. - EMA/OD/057/18

Treatment of acute myeloid leukaemia

Action: For information

Document(s) tabled:
Withdrawal request of 28 June 2018

Notes: There have been 54 designations for this condition: See 2.1.3.

2.1.9. - EMA/OD/044/18

Treatment of hematopoietic stem cell transplant-associated thrombotic microangiopathy

Action: For adoption, Oral explanation to be held on 18 July 2018 at 15:00

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

2.1.10. - EMA/OD/063/18

Treatment of ovarian cancer

Action: For adoption, Oral explanation to be held on 18 July 2018 at 16:00

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

Notes: There have been 30 designations for this condition: EMEA/OD/019/02 Oregovomab, EMEA/OD/061/06 Paclitaxel (micellar), EMEA/OD/080/03 Anti-epithelial cell adhesion molecule/anti-CD3 monoclonal antibody, EMEA/OD/044/03 Trabectedin, EMEA/OD/065/05 Imexon, EMEA/OD/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/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 N-[4-[[(2-amino-3,4-dihydro-4-oxo-6-pteridinyl)methyl]amino]benzoyl]-L-γ-glutamyl-L-α-aspartyl-L-arginyl-L-α-aspartyl-L-α-aspartyl-L-cysteine, EMA/OD/002/12 1-(4-{4-amino-7-[1-(2-hydroxyethyl)-1H- pyrazol-4-yl] thieno [3,2-c]pyridin-3-yl}phenyl)-3-(3-fluorophenyl)urea, EMA/OD/114/12 Alisertib, EMA/OD/314/14 {2-amino-8-[4-(pyrrolidinylcarbonyl)phenyl]-(3H-benzo[f]azepin-4-yl)}-N,N-dipropylcarboxamide

2.1.11. - EMA/OD/060/18

Treatment of glioma

Action: For adoption, Oral explanation to be held on 18 July 2018 at 17:00

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

Notes: There have been 44 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

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

2.1.12. - EMA/OD/066/18

Treatment of heregulin-positive non-small cell lung cancer

Action: For information

Document(s) tabled:
Withdrawal request of 29 June 2018

2.1.13. - EMA/OD/037/18

Treatment of idiopathic pulmonary fibrosis

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

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

Notes: There have been 11 designations for this condition: See 2.1.7.

2.1.14. - EMA/OD/071/18

Treatment of marginal zone lymphoma

Action: For adoption

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

Notes: There have been 2 designations for this condition: EMA/OD/284/14 Lenalidomide, EMA/OD/082/15 Ibrutinib
Designation withdrawn: EMA/OD/014/15 obinutuzumab

2.1.15. - EMA/OD/070/18

Treatment of pulmonary arterial hypertension

Action: For information

Document(s) tabled:
Withdrawal request of 27 June 2018

Notes: There have been 7 designations for this condition: EMEA/OD/018/08 Beraprost sodium, EMA/OD/023/11 Macitentan, EMA/OD/111/11 Sodium nitrite, EMA/OD/179/15 Ubenimex, EMA/OD/299/16 (S)-8-{2-amino-6-[1-(5-chloro-biphenyl-2-yl)-(R)-2,2,2-trifluoro-ethoxy]-pyrimidin-4-yl}-2,8-diaza-spiro[4.5]decane-3-carboxylic acid ethyl ester), EMA/OD/099/17 Tacrolimus, EMA/OD/199/17 N-(tert-butylcarbamoyl)-5-cyano-2-((4'-(difluoromethoxy)-[1,1'-biphenyl]-3-yl)oxy)benzenesulfonamide

2.2. For discussion / preparation for an opinion

2.2.1. - EMA/OD/099/18

Treatment of beta thalassaemia intermedia and major

Action: For discussion/adoption

Document(s) tabled:
Draft Summary report

2.2.2. - EMA/OD/075/18

Treatment of idiopathic pulmonary fibrosis

Action: For discussion/adoption

Document(s) tabled:
Draft Summary report

Notes: There have been 11 designations for this condition: See 2.1.7.

2.2.3. - EMA/OD/095/18

Treatment of spinocerebellar ataxia

Action: For discussion/adoption

Document(s) tabled:
Draft Summary report

Notes: There have been 3 designations for this condition: EMA/OD/216/14 Ceftriaxone, EMA/OD/009/15 Trehalose, EMA/OD/209/16 Trans-resveratrol

2.2.4. - EMA/OD/049/18

Treatment of glioma

Action: For discussion/adoption

Document(s) tabled:
Draft Summary report

Notes: There have been 44 designations for this condition: See 2.1.11.

2.2.5. - EMA/OD/079/18

Treatment of mastocytosis

Action: For discussion/adoption

Document(s) tabled:
Draft Summary report

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

2.2.6. - EMA/OD/098/18

Treatment of bullous pemphigoid

Action: For discussion/adoption

Document(s) tabled:
Draft Summary report

Notes: There has been 1 designation for this condition: EMA/OD/029/16 Dimethyl fumarate

2.2.7. - EMA/OD/088/18

Prevention of Graft Rejection Following Solid Organ Transplantation

Action: For discussion/adoption

Document(s) tabled:
Draft Summary report

Notes: There have been 4 designations for this condition: EMA/OD/308/14 Recombinant monoclonal IgG1 antibody against T-cell immune response cDNA 7, EMA/OD/043/13 Autologous regulatory T cells with an immunophenotype of CD4+CD25hiFoxP3+, EMA/OD/168/13 Ex vivo cultured human mesenchymal stromal cells, EMA/OD/237/16 Recombinant IgG degrading enzyme of Streptococcus pyogenes
Designations withdrawn: EMA/OD/165/12 Murine IgM monoclonal antibody binding to alpha beta T-Cell receptor, EMA/OD/176/13 Eculizumab

2.2.8. - EMA/OD/087/18

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

Action: For discussion/adoption

Document(s) tabled:
Draft Summary report

2.2.9. - EMA/OD/090/18

Treatment of Fanconi anemia

Action: For discussion/adoption

Document(s) tabled:
Draft Summary report

2.2.10. - EMA/OD/089/18

Treatment of neuronal ceroid lipofuscinosis

Action: For discussion/adoption

Document(s) tabled:
Draft Summary report

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

2.2.11. - EMA/OD/094/18

Treatment of acute myeloid leukaemia

Action: For discussion/adoption

Document(s) tabled:
Draft Summary report

Notes: There have been 54 designations for this condition: See 2.1.3.

2.2.12. - EMA/OD/081/18

Prevention of graft-versus-host disease

Action: For discussion/adoption

Document(s) tabled:
Draft Summary report

Notes: There have been 6 designations for this condition: EMEA/OD/054/06 Recombinant fusion protein consisting of the extracellular portion of CD95 fused to the Fc part of a human IgG1 molecule, EMEA/OD/121/07 Donor lymphocyte preparation depleted of functional alloreactive T-cells, EMA/OD/103/13 Defibrotide, EMA/OD/146/13 Allogeneic bone-marrow derived ex-vivo expanded multipotent adult progenitor cells, EMA/OD/163/14 Allogeneic bone marrow derived mesenchymal cells expanded ex vivo in synthetic media, EMA/OD/119/15 Humanised fusion protein consisting of extracellular domain of CD24 linked to IgG1 Fc domain
Designation withdrawn: EMA/OD/131/15 2-amino-2-[2-[2-chloro-4-[[3-(phenylmethoxy)phenyl]thio]phenyl]ethyl]-1,3-propanediol hydrochloride

2.2.13. - EMA/OD/042/18

Treatment of osteosarcoma

Action: For discussion/adoption

Document(s) tabled:
Draft Summary report

Notes: There have been 3 designations for this condition: EMEA/OD/013/04 Muramyl Tripeptide Phosphatidyl Ethanolamine, EMA/OD/020/13 Lipid-complexed cisplatin, EMA/OD/162/15 Live attenuated Listeria monocytogenes bioengineered with a chimeric human epidermal growth factor receptor 2 fused to a truncated form of the Lm protein listeriolysin O
Designation withdrawn: EMEA/OD/004/07 5(S)-(2'-hydroxy ethoxy)-20(S)-Camptothecin

2.2.14. - EMA/OD/077/18

Treatment of acute radiation syndrome

Action: For discussion/adoption

Document(s) tabled:
Draft Summary report

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

2.2.15. - EMA/OD/074/18

Treatment of polycythemia vera

Action: For discussion/adoption

Document(s) tabled:
Draft Summary report

2.2.16. - EMA/OD/080/18

Treatment of inhalational anthrax

Action: For discussion/adoption

Document(s) tabled:
Draft Summary report

2.2.17. - EMA/OD/038/18

Treatment of biliary tract cancer

Action: For discussion/adoption

Document(s) tabled:
Draft Summary report

Notes: There have been 5 designations for this condition: EMA/OD/199/13 (5R,5aR,8aR,9S)-9-[[4,6-O-[(R)-Ethylidene]- Ω-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

2.2.18. - EMA/OD/091/18

Treatment of multiple myeloma

Action: For discussion/adoption

Document(s) tabled:
Draft Summary report

Notes: There have been 16 designations for this condition: EMEA/OD/040/01 Thalidomide, EMEA/OD/044/04 Aplidine, EMEA/OD/066/04 Recombinant histidine-tagged idiotype immunoglobulin Fab fragment of clonal B-cell receptors, EMEA/OD/012/05 N-(methyl-diazacyclohexyl-methylbenzamide)-azaphenyl-aminothiopyrrole, EMEA/OD/120/07 Carfilzomib, EMEA/OD/068/08 N2'-Deacetyl-N2'-[4-methyl-4-(oxobuthyldithio)-1-oxopentyl]-maytansine-chimerized anti-CD138 IgG4 monoclonal antibody, EMEA/OD/076/08 Human anti-intercellular adhesion molecule-1 monoclonal antibody, EMEA/OD/053/08 Milatuzumab, EMEA/OD/053/09 Pomalidomide, EMA/OD/017/11

Acadesine, EMA/OD/048/11 2,2'-{2-[(1R)-1-({[(2,5-dichlorobenzoyl)amino]acetyl}amino)-3-methylbutyl]-5-oxo-1,3,2-dioxaborolane-4,4-diyl}diacetic acid, EMA/OD/113/12 Panobinostat, EMA/OD/125/17 Autologous ex-vivo-expanded peripheral polyclonal lymphocytes enriched in activated natural killer cells, EMA/OD/121/16 Venetoclax, EMA/OD/270/16 Autologous T lymphocyte-enriched population of cells transduced with a lentiviral vector encoding a chimeric antigen receptor targeting human B cell maturation antigen with 4-1BB and CD3-zeta intracellular signalling domains, EMA/OD/077/17 Humanised monoclonal antibody targeting B-cell maturation antigen conjugated with maleimidocaproyl monomethyl auristatin F
Designations withdrawn: EMEA/OD/048/00 Arsenic trioxide, EMEA/OD/003/01 Humanised anti-HM1.24 monoclonal antibody, EMEA/OD/018/00 Thalidomide, EMEA/OD/026/01 Deoxyribose phosphorothioate (5'-tct-ccc-agc-gtg-cgc-cat-3'), EMEA/OD/019/01 Thalidomide, EMEA/OD/070/04 17-allylamino-17-demethoxygeldanamycin, EMEA/OD/093/05 Human monoclonal antibody against HLA-DR, EMEA/OD/003/09 Chimeric-anti-interleukin-6 monoclonal antibody, EMEA/OD/133/09 Dexamethasone (40 mg tablet), EMEA/OD/130/09 Perifosine, EMA/OD/115/10 Maytansinoid-conjugated humanised monoclonal antibody against CD56, EMA/OD/137/10 Vorinostat, EMA/OD/137/11 Chimeric monoclonal antibody against kappa myeloma antigen, EMA/OD/061/12 Elotuzumab

2.2.19. - EMA/OD/078/18

Treatment of acute liver failure

Action: For discussion/adoption

Document(s) tabled:
Draft Summary report

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

2.3. 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 17-19 July 2018 COMP meeting

2.7. Evaluation on-going

Eight 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 bronchiolitis obliterans syndrome

Action: For adoption

3.1.2. -

Treatment of glioma

Action: For adoption

3.1.3. -

Treatment of glioma

Action: For adoption

3.1.4. -

Treatment of glioma

Action: For adoption

3.1.5. -

Prevention of graft rejection following solid organ transplantation

Action: For adoption

3.1.6. -

Treatment of transthyretin-mediated amyloidosis

Action: For adoption

3.2. Finalised letters

3.2.1. -

Treatment of Fabry disease

Action: For information

3.2.2. -

Prevention of oral mucositis in head and neck cancer patients undergoing radiation therapy

Action: For information

3.2.3. -

Treatment of congenital hyperinsulinism

Action: For information

3.2.4. -

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

Action: For information

3.2.5. -

Treatment of multiple myeloma

Action: For information

3.2.6. -

Treatment of graft-versus-host disease

Action: For information

3.2.7. -

Treatment of hairy cell leukaemia

Action: For information

3.3. New requests

None

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. Kymriah - tisagenlecleucel – EMEA/H/C/004090

Novartis Europharm Limited;

a) Treatment of diffuse large B-cell lymphoma EMA/OD/087/16, EU/3/16/1745

b) Treatment of B-lymphoblastic leukaemia/lymphoma EMA/OD/187/13, EU/3/14/1266

Action: For adoption, Oral explanation to be held on 17 July 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 June 2018.

4.1.2. YESCARTA - axicabtagene ciloleucel - EMEA/H/C/004480

Kite Pharma EU B.V.

a) Treatment of primary mediastinal large B-cell lymphoma EMA/OD/078/15, EU/3/15/1553

b) Treatment of follicular lymphoma EMA/OD/135/15, EU/3/15/1579

c) Treatment of diffuse large B cell lymphoma EMA/OD/171/14, EU/3/14/1393

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

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

Notes: Status of the procedure at the CHMP/CAT: CHMP/CAT positive opinion adopted in June 2018.

4.1.3. Veyvondi - vonicog alfa – EMA/OD/055/10, EU/3/10/814, EMEA/H/C/004454

Baxalta Innovations GmbH; Treatment of von Willebrand disease

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

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

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

4.1.4. Lenvima - Lenvatinib – Type II variation - EMEA/H/C/003727/II/0011/G, EMA/OD/287/14, EU/3/15/1460

Eisai Ltd; Treatment of hepatocellular carcinoma

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

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

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

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

4.1.5. Cablivi - caplacizumab - EMEA/OD/109/08, EU/3/09/629, EMEA/H/C/004426

Ablynx NV; Treatment of thrombotic thrombocytopenic purpura

Action: For adoption

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

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

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

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

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

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

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

4.2.2. – paclitaxel - EMEA/OD/061/06, EU/3/06/422, EMEA/H/C/004154

Oasmia Pharmaceutical AB; Treatment of ovarian cancer

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

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

4.2.3. - patisiran – EMEA/H/C/004699, EMA/OD/142/10, EU/3/11/857

Alnylam UK Limited; Treatment of familial amyloid polyneuropathy

Action: For adoption, Oral explanation to be held on 19 July 2018 at time 11:30

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

4.2.4. - 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

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

Shire Pharmaceuticals Ireland Limited; Treatment of hereditary angioedema

Action: For discussion

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

4.3. 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

None

5.2. Prior to adoption of CHMP opinion

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

Vertex Pharmaceuticals; Treatment of cystic fibrosis

CHMP rapporteur: Concepcion Prieto Yerro

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

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

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

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

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

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

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

5.2.3. Blincyto (blinatumomab) - Type II variation – EMEA/OD/029/09, EU/3/09/650, EMEA/H/C/003731/II/0011

Amgen Europe BV - The Netherlands; Treatment of acute lymphoblastic leukaemia

CHMP rapporteur: Alexandre Moreau; CHMP co-rapporteur: Daniela Melchiorri

Action: For discussion

Documents tabled:
Draft report on review of OMPD

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

Clovis Oncology UK Limited; Treatment of ovarian cancer

CHMP rapporteur: Jorge Camarero Jiménez

Action: For discussion

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

5.2.5. RAVICTI (GLYCEROL PHENYLBUTYRATE) - Type II variation – EMEA/H/C/003822/II/0019

Horizon Pharma Ireland Limited;

a) Treatment of ornithine carbamoyltransferase deficiency EMA/OD/002/10, EU/3/10/734

b) Treatment of citrullinaemia type 1 EMA/OD/003/10, EU/3/10/735

c) Treatment of argininosuccinic aciduria EMA/OD/004/10, EU/3/10/736

d) Treatment of hyperargininaemia EMA/OD/005/10, EU/3/10/737

e) Treatment of ornithine translocase deficiency (hyperornithinaemia-hyperammonaemia homocitrullinuria (HHH) syndrome) EMA/OD/006/10, EU/3/10/738

f) Treatment of carbamoyl-phosphate synthase-1 deficiency EMEA/OD/124/09, EU/3/10/733

CHMP rapporteur: Greg Markey

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:
Invitation_COMP-SAWP SR&L Meeting
Practical Information_SAWP-COMP SR&L Meeting

7.1.2. Protocol Assistance Working Group (PAWG)

Proposed meeting time on 17 July 2018 at 13:00

Document tabled:
PAWG draft agenda for 17 June 2018 meeting

7.1.3. Election of COMP Chairperson

Action: For information

Document(s) tabled:
2018-09 - COMP - Election of Chairperson - call for expression of interest
2018-09 - COMP - Election of Chairperson - Annex 1 procedure

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 June 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 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. EMA Business Pipeline activity and Horizon scanning

Action: For information

Document tabled:
Q2/2018 Update of the Business Pipeline report for the human scientific committees

8.2. EMA relocation to Amsterdam, the Netherlands - update

Action: For discussion

Document tabled:
Presentation

8.3. EMA-EUnetHTA bilateral

Scope: Significant Benefit (SB) and Relative Effectiveness Assessment (REA) for Orphan Medicinal Products (OMP)

Action: For discussion

Document tabled:
ppt EMA-EUnetHTA bilateral 5 July 2018

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/