18 June 2018
EMA/COMP/355979/2018
Inspections, Human Medicines Pharmacovigilance and Committees Division

Committee for Orphan Medicinal Products (COMP)

Draft agenda for the meeting on 19-21 June 2018

Chair: Bruno Sepodes – Vice-Chair: Lesley Greene

19 June 2018, 09:00-19:30, room 2F

20 June 2018, 08:30-19:30, room 2F

21 June 2018, 08:30-16:00, 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 19-21 June 2018. See June 2018 COMP minutes (to be published post July 2018 COMP meeting).

1.2. Adoption of agenda

COMP agenda for 19-21 June 2018.

1.3. Adoption of the minutes

COMP minutes for 22-24 May 2018.

2. Applications for orphan medicinal product designation

2.1. For opinion

2.1.1. - EMA/OD/253/17

Treatment of pilonidal sinus disease

Action: For information

Document(s) tabled:
Withdrawal request of 5 June 2018

2.1.2. - EMA/OD/024/18

Treatment in haematopoietic stem cell transplantation

Action: For adoption, Oral explanation to be held on 19 June 2018 at 14:30

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

Notes: There have been 8 designations for this condition: EMA/OD/008/16 Donor T lymphocytes depleted ex vivo of host alloreactive T cells using photodynamic treatment, EMA/OD/020/16 Allogeneic donor-derived ex-vivo expanded T lymphocytes transduced with a retroviral vector containing inducible caspase 9 and truncated CD19, EMA/OD/090/16 Radio-iodinated (131I) anti-CD45 murine monoclonal antibody, EMA/OD/149/16 Allogeneic peripheral blood mononuclear cells incubated ex vivo with 16, 16-dimethyl prostaglandin E2 and dexamethasone, EMA/OD/191/16 Human donor haematopoietic stem and progenitor cells that have been treated ex vivo with the protein transduction domain of the HIV-1 transactivation protein fused to MYC transcription factor, EMA/OD/257/16 Allogeneic, ex vivo expanded, umbilical cord blood-derived, hematopoietic CD34+ progenitor cells and allogeneic, non-expanded, umbilical cord blood-derived, hematopoietic mature myeloid and lymphoid cells, EMA/OD/150/17 Allogeneic CD4+ and CD25+ T lymphocytes ex vivo

incubated with GP120, EMA/OD/192/17 Allogeneic umbilical cord blood CD34+ cells cultured ex vivo with Notch ligand Delta1

2.1.3. - EMA/OD/036/18

Treatment of glioma

Action: For adoption, Oral explanation to be held on 19 June 2018 at 15:30

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

Notes: There have been 43 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)m 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

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

2.1.4. - EMA/OD/030/18

Treatment of idiopathic pulmonary fibrosis

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

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

2.1.5. - EMA/OD/026/18

Treatment of acute myeloid leukaemia

Action: For adoption, Oral explanation to be held on 20 June 2018 at 15:30

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 di-hydrochloride salt, EMEA/OD/028/09 Tosedostat, EMEA/OD/091/09 1-Cyclopropyl-3-[3-(5-morpholin-4-ylmethyl-1H-benzoimidazol-2-yl)-1H-pyrazol-4-yl]-urea, EMEA/OD/147/09 2-methoxymethyl-2-hydroxymethyl-1-azabicyclo[2,2,2]octan-3-one, EMA/OD/044/10 Allogeneic T cells encoding an exogenous TK gene, EMA/OD/094/10 N-[(2S)-2,3-dihydroxypropyl]-3-[(2-fluoro-4-iodophenyl) amino] isonicotinamide hydrochloride, EMA/OD/101/11 Allogeneic human dendritic cells derived from a CD34+ progenitor cell line, EMA/OD/070/11 Liposomal combination of cytarabine and daunorubicin, EMA/OD/158/11 Vosaroxin, EMA/OD/167/12 L-asparaginase encapsulated in erythrocytes, EMA/OD/064/13 trans-N1-((1R,2S)-2-phenylcyclopropyl)cyclohexane-1,4-diamine bis-hydrochloride, EMA/OD/141/13 (2R,3R,4S,5R)-2-(6-amino-9H-purin-9-yl)-5-((((1r,3S)-3-(2-(5-(tert-butyl)-1Hbenzo[d]imidazol-2-yl)ethyl)cyclobutyl)(isopropyl) amino)methyl) tetrahydrofuran-3,4-diol, EMA/OD/181/13 Volasertib, EMA/OD/100/14 4-{[(2R,3S,4R,5S)-4-(4-Chloro-2-fluoro-phenyl)-3-(3-chloro-2-fluoro-phenyl)-4-cyano-5-(2,2-dimethyl-propyl)-pyrrolidine-2-carbonyl]-amino}-3-methoxy-benzoic acid, EMA/OD/061/14 (Z)-3-(3-(3,5-bis(trifluoromethyl)phenyl)-1H-1,2,4-triazol-1-yl)-N'-(pyrazin-2-yl)acrylohydrazide, EMA/OD/103/14 Donor T lymphocytes depleted ex vivo of host alloreactive T cells using photodynamic treatment, EMA/OD/175/14 Allogeneic ex vivo-generated natural killer cells from CD34+ umbilical cord blood progenitor cells, EMA/OD/240/14 Alvocidib, EMA/OD/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), EMEA/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, EMEA/OD/161/10 Allogeneic bone marrow stem cells treated ex vivo with 16,16-dimethyl prostaglandin E2, EMEA/OD/156/10 Allogeneic umbilical cord blood cells treated ex vivo with 16,16-dimethyl prostaglandin E2, EMEA/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, EMEA/OD/105/12 Liposomal daunorubicin, EMEA/OD/188/14 Allogeneic, umbilical cord blood-derived, ex vivo-expanded, haematopoietic CD133+ cells / allogeneic, umbilical cord blood-derived, non-expanded, haematopoietic CD133- cells

2.1.6. - EMA/OD/032/18

Treatment of Duchenne muscular dystrophy

Action: For adoption, Oral explanation to be held on 20 June 2018 at 11:00

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

Notes: There have been 27 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, 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

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

2.1.7. - EMA/OD/108/17

Treatment of abdominal aortic aneurysm

Action: For adoption, Oral explanation to be held on 20 June 2018 at 12:00

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

2.1.8. - EMA/OD/034/18

Treatment of epidermolysis bullosa

Action: For information

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

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

2.1.9. - EMA/OD/028/18

Treatment of Duchenne muscular dystrophy

Action: For adoption

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

Notes: There have been 27 designations for this condition: See 2.1.6.

2.2. For discussion / preparation for an opinion

2.2.1. - EMA/OD/060/18

Treatment of glioma

Action: For discussion/adoption

Document(s) tabled:
Draft Summary report

Notes: There have been 43 designations for this condition: See 2.1.3.

2.2.2. - EMA/OD/058/18

Treatment of progressive supranuclear palsy

Action: For discussion/adoption

Document(s) tabled:
Draft Summary report

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

2.2.3. - EMA/OD/051/18

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 2.1.5.

2.2.4. - EMA/OD/050/18

Treatment of behavioural variant frontotemporal dementia

Action: For discussion/adoption

Document(s) tabled:
Draft Summary report

Notes: There have been 2 designations for this condition: EMA/OD/063/10 Methylthioninium, EMA/OD/194/15 Tolfenamic acid

2.2.5. - EMA/OD/065/18

Treatment of spinal muscular atrophy

Action: For discussion/adoption

Document(s) tabled:
Draft Summary report

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

2.2.6. - EMA/OD/048/18

Treatment of achromatopsia

Action: For discussion/adoption

Document(s) tabled:
Draft Summary report

2.2.7. - EMA/OD/057/18

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 2.1.5.

2.2.8. - EMA/OD/070/18

Treatment of pulmonary arterial hypertension

Action: For discussion/adoption

Document(s) tabled:
Draft Summary report

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

2.2.9. - EMA/OD/046/18

Treatment of eosinophilic oesophagitis

Action: For discussion/adoption

Document(s) tabled:
Draft Summary report

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

2.2.10. - EMA/OD/072/18

Treatment of follicular lymphoma

Action: For discussion/adoption

Document(s) tabled:
Draft Summary report

Notes: There have been 10 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/222/17 Tazemetostat

Designations withdrawn: EMEA/OD/061/02 Iodine (131I) tositumomab, EMEA/OD/079/02 Tositumomab, EMA/OD/053/13 Idelalisib

2.2.11. - EMA/OD/071/18

Treatment of marginal zone lymphoma

Action: For discussion/adoption

Document(s) tabled:
Draft Summary report

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

2.2.12. - EMA/OD/023/18

Treatment of graft-versus-host disease

Action: For discussion/adoption

Document(s) tabled:
Draft Summary report

Notes: There have been 13 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
Designations withdrawn: EMEA/OD/020/00 Thalidomide, EMA/OD/118/16 Recombinant humanised monoclonal antibody against human complement component C5a

2.2.13. - EMA/OD/062/18

Treatment of Becker muscular dystrophy

Action: For discussion/adoption

Document(s) tabled:
Draft Summary report

Notes: There has been 1 designation for this condition: EMA/OD/032/12 Ataluren

2.2.14. - EMA/OD/056/18

Treatment of vanishing white matter

Action: For information

Document(s) tabled:

Withdrawal request of 14 June 2018

2.2.15. - EMA/OD/055/18

Treatment of Phosphomannomutase 2-Congenital Disorder of Glycosylation (PMM2-CDG)

Action: For discussion/adoption

Document(s) tabled:
Draft Summary report

2.2.16. - EMA/OD/063/18

Treatment of ovarian cancer

Action: For discussion/adoption

Document(s) tabled:
Draft Summary report

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

2.2.17. - EMA/OD/061/18

Treatment of congenital alpha-1 antitrypsin deficiency

Action: For discussion/adoption

Document(s) tabled:
Draft Summary report

Notes: There have been 2 designations for this condition: EMEA/OD/078/06 Recombinant adeno-associated viral vector containing human alpha-1 antitrypsin gene, EMEA/OD/001/08 Alpha-1 proteinase inhibitor (for inhalation use)
Designation withdrawn: EMA/OD/178/15 Synthetic double-stranded oligomer specific to the SERPINA1 gene and containing a cholesterol-conjugated, acyclic nucleobase analogue

2.2.18. - EMA/OD/037/18

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 2.1.4.

2.2.19. - EMA/OD/053/18

Treatment of ectonucleotide pyrophosphatase/ phosphodiesterase 1 deficiency

Action: For discussion/adoption

Document(s) tabled:
Draft Summary report

2.2.20. - EMA/OD/044/18

Treatment of hematopoietic stem cell transplant-associated thrombotic microangiopathy

Action: For discussion/adoption

Document(s) tabled:
Draft Summary report

2.2.21. - EMA/OD/045/18

Treatment of neurofibromatosis type 1

Action: For discussion/adoption

Document(s) tabled:
Draft Summary report

2.2.22. - EMA/OD/066/18

Treatment of heregulin-positive non-small cell lung cancer

Action: For discussion/adoption

Document(s) tabled:
Draft Summary report

2.2.23. - EMA/OD/041/18

Treatment of growth hormone deficiency

Action: For discussion/adoption

Document(s) tabled:
Draft Summary report

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

2.2.24. - EMA/OD/052/18

Treatment of Primary Hyperoxaluria

Action: For discussion/adoption

Document(s) tabled:
Draft Summary report

Notes: There have been 2 designations for this condition: EMEA/OD/095/05 Oxalobacter formigenes strain HC-1, EMA/OD/058/17 Bacillus subtilis oxalate decarboxylase

2.2.25. - EMA/OD/043/18

Treatment of Duchenne muscular dystrophy

Action: For discussion/adoption

Document(s) tabled:
Draft Summary report

Notes: There have been 27 designations for this condition: See 2.1.6.

2.2.26. - EMA/OD/039/18

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 2.1.4.

2.3. Revision of the COMP opinions

None

2.4. Amendment of existing orphan designations

None

2.5. Appeal

None

2.6. Nominations

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

Action: For adoption

Document tabled:
OMPD applications - appointment of coord. at the 19-21 June 2018 COMP meeting

2.7. Evaluation on-going

Four applications for orphan designation will not be discussed as evaluation is on-going. **Action**: For information

Notes: See 7.8.1. Table 6. Evaluation Ongoing.

3. Requests for protocol assistance with significant benefit question

3.1. Ongoing procedures

3.1.1. -

Treatment of Fabry disease

Action: For adoption

3.1.2. -

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

Action: For adoption

3.1.3. -

Treatment of congenital hyperinsulinism

Action: For adoption

3.1.4. -

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

Action: For adoption

3.1.5. -

Treatment of multiple myeloma

Action: For adoption

3.1.6. -

Treatment of bronchiolitis obliterans syndrome

Action: For adoption

3.1.7. -

Treatment of graft-versus-host disease

Action: For adoption

3.1.8. -

Treatment of hairy cell leukaemia

Action: For adoption

3.2. Finalised letters

3.2.1. -

Treatment of acute myeloid leukaemia

Action: For information

3.2.2. -

Treatment of pemphigus

Action: For information

3.2.3. -

Treatment of small cell lung cancer

Action: For information

3.2.4. -

Treatment of tuberous sclerosis

Action: For information

3.2.5. -

Treatment of acute myeloid leukaemia

Action: For information

3.3. New requests

3.3.1. -

Treatment of glioma

Action: For information

3.3.2. -

Treatment of glioma

Action: For information

3.3.3. -

Treatment of glioma

Action: For information

3.3.4. -

Prevention of graft rejection following solid organ transplantation

Action: For information

3.3.5. -

Treatment of transthyretin-mediated amyloidosis

Action: For information

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

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

None

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

4.2.1. – daunorubicin/ cytarabine - EMEA/H/C/004282, EMA/OD/070/11, EU/3/11/942

Jazz Pharmaceuticals Ireland Limited; Treatment of adults with high-risk acute myeloid leukaemia (AML)

Action: For discussion

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

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

Alnylam UK Limited; Treatment of familial amyloid polyneuropathy

Action: For discussion

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

4.2.3. - 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 discussion

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

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

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

Action: For discussion

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

4.2.5. - volanesorsen – EMEA/H/C/004538, EMA/OD/180/13, EU/3/14/1249

Akcea Therapeutics UK Ltd; Treatment of familial chylomicronemia syndrome

Action: For discussion

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

4.2.6. - 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 discussion

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

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

Oasmia Pharmaceutical AB; Treatment of ovarian cancer

Action: For discussion

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

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

Eisai Ltd; Treatment of hepatocellular carcinoma

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

Action: For information

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

4.3. 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

None

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 discussion

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 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

7.1.2. Protocol Assistance Working Group (PAWG)

Proposed meeting time on 19 June 2018 at 13:00

Document tabled:
PAWG draft agenda for 19 June 2018 meeting

7.1.3. Non-Clinical Working Group

Proposed meeting time on 20 June 2018 at 08:30

7.1.4. Condition Working Group

Proposed meeting time on 21 June 2018 at 08:30

7.1.5. Prevalence Working Group

Proposed meeting time on 20 June 2018 at 13:00

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 May 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

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

Action: For information

7.4. Cooperation within the EU regulatory network

7.4.1. European Commission

None

7.4.2. Priority needs and plans for training 2018 – 2020

Scope: Understanding the training needs of members / experts involved in the work of the COMP

Action: For discussion

7.5. Cooperation with International Regulators

7.5.1. Food and Drug Administration (FDA)

Action: For information

Notes: Monthly teleconference

7.5.2. Japanese Pharmaceuticals and Medical Devices Agency (PMDA)

Action: For information

Notes: Ad hoc basis meeting

7.5.3. The Therapeutic Goods Administration (TGA), Australia

Action: For information

Notes: Ad hoc basis meeting

7.5.4. Health Canada

Action: For information

Notes: Ad hoc basis meeting

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

None

7.7. COMP work plan

None

7.8. Planning and reporting

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

Action: For information

7.8.2. Overview of orphan marketing authorisations/applications

Action: For information

8. Any other business

None

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/