2 November 2016
EMA/COMP/668959/2016
Inspections, Human Medicines Pharmacovigilance and Committees Division

Committee for Orphan Medicinal Products (COMP)

Draft agenda for the meeting on 03-04 November 2016

Chair: Bruno Sepodes – Vice-Chair: Lesley Greene

03 November 2016, 08:30-20:00, room 2F

04 November 2016, 08:30-15:30, room 2F

Health and safety information

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

Disclaimers

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

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

Note on access to documents

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

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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 3-4 November 2016. See November 2016 COMP minutes (to be published post December 2016 COMP meeting).

1.2. Adoption of agenda

COMP agenda for 3-4 November 2016.

1.3. Adoption of the minutes

COMP minutes for 4-6 October 2016.

2. Applications for orphan medicinal product designation

2.1. For opinion

2.1.1. - EMA/OD/170/16

Treatment of gastric cancer

Action: For adoption, Oral explanation to be held on 4 November 2016 at time 10:00

Documents tabled:
Draft Summary report with response to LoQs

Notes:
There have been 5 designations for this condition: EMEA/OD/056/02 G17(9) gastrin-Diphtheria Toxoid Conjugate, EMEA/OD/044/06 Catumaxomab, EMA/OD/083/10 Chimeric monoclonal antibody against claudin-18 splice variant 2, EMA/OD/101/10 Tesetaxel, EMA/OD/012/14 Rilotumumab

Designations withdrawn: EMEA/OD/073/07 Tegafur, gimeracil, oteracil potassium, EMA/OD/022/11 Everolimus, EMA/OD/030/12 Ramucirumab

2.1.2. - EMA/OD/134/16

Treatment of multiple myeloma

Action: For information

Documents tabled:
Withdrawal request of 25 October 2016

Notes:

There have been 13 designations for this condition: EMEA/OD/040/01 Thalidomide, EMEA/OD/063/03 3-(4'aminoisoindoline-1'-one)-1-piperidine-2,6-dione, 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

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

2.1.3. - EMA/OD/165/16

Treatment of retinitis pigmentosa

Action: For adoption, Oral explanation to be held on 3 November 2016 at time 11:00

Documents tabled:
Draft Summary report with response to LoQs

Notes:
There have been 17 designations for this condition: EMEA/OD/057/06 4,7,10,13,16,19-Docosahexaenoic acid, EMEA/OD/043/07 Adenovirus associated viral vector serotype 4 containing the human RPE65 gene, EMEA/OD/087/08 Recombinant human proinsulin, EMA/OD/162/10 9-cis-Retinyl acetate, EMA/OD/159/11 Encapsulated human retinal pigment epithelial cell line transfected with plasmid vector expressing human ciliary neurotrophic factor, EMA/OD/006/12 Recombinant human methionine proinsulin, EMA/OD/025/13 Expanded human allogeneic neural retinal progenitor cells extracted from neural retina, EMA/OD/015/13 Recombinant human nerve growth factor, EMA/OD/031/13 Adenovirus associated viral vector serotype 5 containing the human pde6β gene, EMA/OD/289/14 Sodium 3-[(4aR,6R,7R,7aS)-7-hydroxy-2-oxido-2-sulfanylidene-4a,6,7,7a-tetrahydro-4H-furo[3,2-d][1,3,2]dioxaphosphinin-6-yl]-2-bromo-6-phenyl-5H-imidazo[1,2-a]purin-9-one, EMA/OD/271/14 Myriocin, EMA/OD/327/14 Recombinant human mesencephalic astrocyte-derived neurotrophic factor, EMA/OD/040/15 Adenovirus-associated viral vector serotype 2 containing the human RPE65 gene, EMA/OD/213/15 Allogeneic fetal human retinal progenitor cells expanded ex vivo, EMA/OD/208/15 4-[(2E)-1-oxo-3-(2,6,6-trimethyl-1-cyclohexen-1-yl)-2-propen-1-yl]-1-piperazinecarboxamide, EMA/OD/028/16 Adeno-associated viral vector serotype 2.7m8 containing the ChrimsonR-tdTomato gene, EMA/OD/102/16 Adenovirus associated viral vector serotype 5 containing the human RPGR gene

Designations withdrawn: EMEA/OD/075/07 Recombinant human rod-derived cone viability factor, EMEA/OD/106/07 Allogeneic human umbilical cord tissue-derived cells, EMA/OD/021/12 17-(Dimethylaminoethylamino)-17-demethoxygeldanamycin (after administration of adeno-associated viral vector encoding an inducible short hairpin RNA targeting claudin-5), EMA/OD/135/12 Adeno-associated viral vector encoding an inducible short hairpin RNA targeting claudin-5 (prior to administration of 17-dimethylaminoethylamino-17-demethocygeldanamycin), EMA/OD/067/13 Unoprostone isopropyl

2.1.4. - EMA/OD/166/16

Treatment of angiosarcoma

Action: For adoption, Oral explanation to be held on 3 November 2016 at time 12:00

Documents tabled:
Draft Summary report with response to LoQs

2.1.5. - EMA/OD/188/16

Treatment of pulmonary arterial hypertension

Action: For adoption, Oral explanation to be held on 3 November 2016 at time 14:00

Documents tabled:
Draft Summary report with response to LoQs

Notes:
There have been 3 designations for this condition: EMEA/OD/018/08 Beraprost sodium, EMA/OD/023/11 Macitentan, EMA/OD/111/11 Sodium nitrite

2.1.6. - EMA/OD/163/16

Treatment of congenital adrenal hyperplasia

Action: For adoption, Oral explanation to be held on 3 November 2016 at time 15:00

Documents tabled:
Draft Summary report with response to LoQs

Notes:
There has been 1 designation for this condition: EMEA/OD/020/05 Hydrocortisone (modified release tablet)

Designation withdrawn: EMA/OD/063/15 Verucerfont

2.1.7. - EMA/OD/062/16

Treatment of poisoning by local anesthetics

Action: For adoption, Oral explanation to be held on 3 November 2016 at time 16:30

Documents tabled:
Draft Summary report with response to LoQs

2.1.8. - EMA/OD/181/16

Treatment of plasma cell myeloma

Action: For information

Documents tabled:
Withdrawal request of 19 October 2016

Notes:
There have been 8 designations for this condition: EMA/OD/072/13 Recombinant human monoclonal IgM antibody targeting glucose-regulated protein 78, EMA/OD/038/13 Daratumumab, EMA/OD/198/13 humanized monoclonal antibody against CD38, EMA/OD/035/14 Marizomib, EMA/OD/087/14 Selinexor, EMA/OD/293/14 Melphalan flufenamide, EMA/OD/214/14 Synthetic signal peptide of human Mucin-1 (amino acids 1-21), EMA/OD/277/14 Reduced oxidised N-acetyl heparin

2.1.9. - EMA/OD/169/16

Treatment of tenosynovial giant cell tumour, localised and diffuse type

Action: For adoption, Oral explanation to be held on 3 November 2016 at time 18:30

Documents tabled:
Draft Summary report with response to LoQs

Notes:
There has been 1 designation for this condition: EMA/OD/279/14 [5-(5-chloro-1H-pyrrolo[2,3-b]pyridin-3-ylmethyl)-pyridin-2-yl]-(6-trifluoromethyl-pyridin-3-ylmethyl)-amine hydrochloride

2.1.10. - EMA/OD/152/16

Treatment of Huntington's disease

Action: For information

Documents tabled:
Withdrawal request of 18 October 2016

Notes:
There have been 6 designations for this condition: EMA/OD/070/14 Cysteamine bitartrate, EMA/OD/169/14 5-bromo-N-(prop-2-yn-1-yl)-2-(1H-1,2,4-triazol-1-yl) pyrimidine-4,6-diamine, EMA/OD/256/14 Chimeric 2'-O-(2-methoxyethyl) modified oligonucleotide targeted to huntingtin RNA, EMA/OD/311/14 Phenol, 4-[2-(aminomethyl)-4-thiazolyl]-2,6-bis(1,1-dimethylethyl) monohydrochloride, EMA/OD/325/14 AASSGVSTPGSAGHDIITEQPRS, EMA/OD/017/15 5,7-dichloro-2-dimethylaminomethyl-8-hydroxyquinoline hydrochloride

2.1.11. - EMA/OD/177/16

Treatment of recurrent Clostridium difficile infection

Action: For adoption, Oral explanation to be held on 4 November 2016 at time 11:00

Documents tabled:

Draft Summary report with response to LoQs

2.1.12. - EMA/OD/208/16

Treatment of graft versus host disease

Action: For adoption

Documents tabled:
Draft Summary report with response to LoQs

Notes:
There have been 7 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, EMA/OD/131/15 2-amino-2-[2-[2-chloro-4-[[3-(phenylmethoxy)phenyl]thio]phenyl]ethyl]-1,3-propanediol hydrochloride

2.2. For discussion / preparation for an opinion

2.2.1. - EMA/OD/197/16

Treatment of acute myeloid leukaemia

Action: For adoption

Documents tabled:
Draft Summary report

Notes:
There have been 48 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/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/101/11 Allogeneic human dendritic cells derived from a CD34+ progenitor cell line, EMA/OD/070/11 Liposomal combination of cytarabine and daunorubicin, EMA/OD/158/11 Vosaroxin, EMA/OD/167/12 L-asparaginase encapsulated in erythrocytes, EMA/OD/064/13 trans-N1-((1R,2S)-2-phenylcyclopropyl)cyclohexane-1,4-diamine bis-hydrochloride, EMA/OD/141/13 (2R,3R,4S,5R)-2-(6-amino-9H-purin-9-yl)-5-((((1r,3S)-3-(2-(5-(tert-butyl)-1Hbenzo[d]imidazol-2-yl)ethyl)cyclobutyl)(isopropyl) amino)methyl)tetrahydrofuran-3,4-diol, EMA/OD/181/13 Volasertib, EMA/OD/100/14 4-{[(2R,3S,4R,5S)-4-(4-Chloro-2-fluoro-phenyl)-3-(3-chloro-2-fluoro-phenyl)-4-cyano-5-(2,2-dimethyl-propyl)-pyrrolidine-2-carbonyl]-amino}-3-methoxy-benzoic acid, EMA/OD/061/14 (Z)-3-(3-(3,5-bis(trifluoromethyl)phenyl)-1H-1,2,4-triazol-1-yl)-N'-(pyrazin-2-yl)acrylohydrazide, EMA/OD/103/14 Donor T lymphocytes depleted ex vivo of host alloreactive T cells using photodynamic treatment, EMA/OD/175/14 Allogeneic ex vivo-generated natural killer cells from CD34+ umbilical cord blood progenitor cells, EMA/OD/240/14 Alvocidib, EMA/OD/188/14 Allogeneic, umbilical cord blood-derived, ex vivo-expanded, haematopoietic CD133+ cells / allogeneic, umbilical cord blood-derived, non-expanded, haematopoietic CD133- cells, EMA/OD/258/14 Ulocuplumab, EMA/OD/045/15 inecalcitol, EMA/OD/037/15 2-((3-((4-((3-aminopropyl)amino)butyl)amino)propyl)amino)-N-((5S,5aS,8aR,9R)-9-(4-hydroxy-3,5-dimethoxyphenyl)-8-oxo-5,5a,6,8,8a,9-hexahydrofuro[3',4':6,7]naphtho[2,3-d][1,3]dioxol-5-yl)acetamide, tetrahydrochloride, EMA/OD/089/15 CD33-directed antibody-drug conjugate consisting of an antibody conjugated to a DNA cross-linking pyrrolobenzodiazepine dimer drug, EMA/OD/112/15 Recombinant human interleukin-3 truncated diphtheria toxin fusion protein, EMA/OD/145/15 Humanised monoclonal antibody of the IgG4 kappa isotype targeting CD47, EMA/OD/165/15 Sodium (2R,3S,5R)-5-(4-amino-2-oxo-1,3,5-triazin-1(2H)-yl)-2-(hydroxymethyl)tetrahydrofuran-3-yl ((2R,3S,5R)-5-(2-amino-6-oxo-1H-purin-9(6H)-yl)-3-hydroxytetrahydrofuran-2-yl)methyl phosphate, EMA/OD/144/15 Combretastatin A1-diphosphate, EMA/OD/180/15 Arsenic trioxide, EMA/OD/205/15 Venetoclax, EMA/OD/233/15 Tyr-Met-Phe-Pro-Asn-Ala-Pro-Tyr-Leu, Ser-Gly-Gln-Ala-Tyr-Met-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

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/067/11 1-(4-{4-amino-7-[1-(2-

hydroxyethyl)-1H- pyrazol-4-yl]thieno[3,2-c]pyridin-3-yl}phenyl)-3-(3-fluorophenyl)urea, EMA/OD/105/12 Liposomal daunorubicin

2.2.2. - EMA/OD/205/16

Treatment of Cockayne syndrome

Action: For adoption

Documents tabled:
Draft Summary report

2.2.3. - EMA/OD/212/16

Treatment of multiple myeloma

Action: For adoption

Documents tabled:
Draft Summary report

Notes:
There have been 13 designations for this condition: EMEA/OD/040/01 Thalidomide, EMEA/OD/063/03 3-(4'aminoisoindoline-1'-one)-1-piperidine-2,6-dione, 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

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

2.2.4. - EMA/OD/215/16

Treatment of glioma

Action: For adoption

Documents tabled:
Draft Summary report

Notes:

There have been 42 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/050/07 Doxorubicin hydrochloride (drug eluting beads), EMEA/OD/051/07 Irinotecan hydrochloride (drug eluting beads), 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/019/12 Doxorubicin (administered after synthetic double-stranded siRNA oligonucleotide directed against claudin-5 complexed with polyethyleneimine), 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/136/12 Synthetic double-stranded siRNA oligonucleotide directed against Claudin-5 complexed with polyethyleneimine (prior to administration of doxorubicin), 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

Designations withdrawn: EMEA/OD/067/01 Carmustine (solution for intratumoral injection), 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/037/02 Iodine (131I) anti-nucleohistone H1 chimeric biotinylated monoclonal antibody, EMEA/OD/067/03 Cilengitide, EMEA/OD/050/06 Iodine (131I) anti-tenascin monoclonal antibody 81C6, 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/113/15 Dronabinol and cannabidiol

2.2.5. - EMA/OD/206/16

Diagnosis of gastrointestinal stromal tumours

Action: For adoption

Documents tabled:
Draft Summary report

2.2.6. - EMA/OD/190/16

Treatment of achromatopsia caused by mutations in the *CNGA3* gene

Action: For adoption

Documents tabled:
Draft Summary report

Notes:
There has been 1 designation for this condition: EMA/OD/096/15 Recombinant adeno-associated viral vector containing the human CNGA3 gene

2.2.7. - EMA/OD/214/16

Treatment of status epilepticus

Action: For adoption

Documents tabled:
Draft Summary report

2.2.8. - EMA/OD/200/16

Treatment of paediatric stroke

Action: For adoption

Documents tabled:
Draft Summary report

2.2.9. - EMA/OD/203/16

Treatment of Rett syndrome

Action: For adoption

Documents tabled:
Draft Summary report

Notes:

There have been 4 designations for this condition: EMEA/OD/001/09 Desipramine chlorhydrate, EMA/OD/163/13 3-Chloro-4-fluorophenyl-[4-fluoro-4-{[(5-methylpyrimidin-2-ylmethyl) amino]methyl}piperidin-1-yl]methanone, EMA/OD/056/15 Glycyl-L-2-methylprolyl-L-glutamic acid, EMA/OD/058/15 Sarizotan hydrochloride

2.2.10. - EMA/OD/202/16

Treatment of Wolfram syndrome

Action: For adoption

Documents tabled:
Draft Summary report

Notes:
There has been 1 designation for this condition: EMA/OD/222/14 sodium valproate

2.2.11. - EMA/OD/213/16

Treatment of antiphospholipid syndrome

Action: For adoption

Documents tabled:
Draft Summary report

2.2.12. - EMA/OD/182/16

Treatment of amyotrophic lateral sclerosis

Action: For adoption

Documents tabled:
Draft Summary report

Notes:
There have been 18 designations for this condition: EMEA/OD/053/06 Arimoclomol, EMEA/OD/102/07 Filgrastim, EMEA/OD/096/08 (6R)-4,5,6,7-tetrahydro-N6-propyl-2,6-benzothiazole-diamine dihydrochloride monohydrate, EMEA/OD/108/09 Recombinant human vascular endothelial growth factor, EMA/OD/043/11 Smilagenin, EMA/OD/106/11 S[+] apomorphine, EMA/OD/138/11 6-ethynyl-1-(pentan-3-yl)-1H-imidazo[4,5-b]pyrazin-2(3H)-one, EMA/OD/011/13 Autologous bone marrow-derived mesenchymal stromal cells secreting neurotrophic factors, EMA/OD/023/13 Sodium chlorite, EMA/OD/044/13 Allogeneic motor neuron progenitor cells derived from human embryonic stem cells, EMA/OD/184/14 Edaravone, EMA/OD/283/14 Enoxacin, EMA/OD/032/15 Edaravone, EMA/OD/051/15 Hydrocinnamate-[Orn-Pro-dCha-Trp-Arg]acetate, EMA/OD/011/16 H-Phe-Ser-Arg-Tyr-Ala-Arg-OH-acetate, EMA/OD/241/15 Recombinant human cerebral dopamine neurotrophic factor, EMA/OD/081/16 Masitinib mesilate, EMA/OD/120/16 Synthetic ribonucleic acid oligonucleotide directed against superoxide dismutase 1 messenger ribonucleic acid

Designations withdrawn: EMEA/OD/029/00 Xaliproden hydrochloride, EMEA/OD/030/06 Cholest-4-en-3-one, oxime, EMEA/OD/125/07 Sarsasapogenin, EMEA/OD/012/09 Talampanel, EMA/OD/060/10 Recombinant humanised monoclonal antibody to human Nogo-A protein of the IgG1/kappa class

2.2.13. - EMA/OD/210/16

Prevention of necrotising enterocolitis

Action: For adoption

Documents tabled:
Draft Summary report

Notes:
There have been 2 designations for this condition: EMA/OD/112/13 Lactobacillus acidophilus / Bifidobacterium bifidum, EMA/OD/237/14 Lactobacillus reuteri

2.2.14. - EMA/OD/097/16

Treatment of primary hypogonadotropic hypogonadism

Action: For adoption

Documents tabled:
Draft Summary report

2.2.15. - EMA/OD/179/16

Treatment of progressive myoclonic epilepsy type 2 (Lafora disease)

Action: For adoption

Documents tabled:
Draft Summary report

2.2.16. - EMA/OD/192/16

Treatment of pulmonary arterial hypertension

Action: For adoption

Documents tabled:
Draft Summary report

Notes:
There have been 3 designations for this condition: EMEA/OD/018/08 Beraprost sodium, EMA/OD/023/11 Macitentan, EMA/OD/111/11 Sodium nitrite

2.2.17. - EMA/OD/193/16

Treatment of pancreatic cancer

Action: For adoption

Documents tabled:
Draft Summary report

Notes:
There have been 33 designations for this condition: EMEA/OD/055/02 G17(9) gastrin-Diphtheria Toxoid Conjugate, EMEA/OD/068/02 Rubitecan, EMA/OD/302/14 Human reovirus

type 3 Dearing strain, 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/150/10 Salirasib, EMA/OD/007/11 Mixture of seven synthetic fragments consisting of p21 RAS peptides, EMA/OD/008/11 Genetically modified human adenovirus encoding human PH20 hyaluronidase, EMA/OD/051/11 Nanoliposomal irinotecan, EMA/OD/065/12 Mixture of two allogeneic human pancreatic cancer cell lines stably transduced with a retroviral vector encoding the murine alpha-(1,3)-galactosyltransferase gene, EMA/OD/037/13 (1-methyl-2-nitro-1H-imidazole-5-yl)methyl N,N'-bis(2-bromoethyl)diamidophosphate, 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 (TEXT TOO LONG), 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/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

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/007/12 Polyinosine-polycytidylic acid coupled with the polycationic polyethyleneimine, EMA/OD/145/12 Recombinant human monoclonal antibody of the IgG1 kappa class against prostate stem cell antigen

2.2.18. - EMA/OD/189/16

Treatment of acute sensorineural hearing loss

Action: For adoption

Documents tabled:
Draft Summary report

2.2.19. - EMA/OD/196/16

Treatment of Guillain-Barré syndrome

Action: For adoption

Documents tabled:
Draft Summary report

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

Designation withdrawn: EMEA/OD/101/06 Fampridine

2.2.20. - EMA/OD/195/16

Treatment of neuronal ceroid lipofuscinosis type 3

Action: For adoption

Documents tabled:
Draft Summary report

2.2.21. - EMA/OD/199/16

Treatment of pancreatic cancer

Action: For adoption

Documents tabled:
Draft Summary report

Notes:
There have been 33 designations for this condition: Please see 2.2.17.

2.2.22. - EMA/OD/218/16

Treatment of functional single ventricle congenital heart disease

Action: For adoption

Documents tabled:
Draft Summary report

2.3. Revision of the COMP opinions

None

2.4. COMP opinions adopted via written procedure following previous meeting

2.4.1. Recombinant human acid sphingomyelinase – EMEA/OD/004/01, EU/3/01/056

Genzyme Europe BV; Treatment of Niemann-Pick disease

COMP coordinator: Pauline Evers

Action: For information

Document tabled:
Amended summary report

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 3-4 November 2016 COMP meeting

2.7. Evaluation on-going

Thirty applications for orphan designation will not be discussed as evaluation is on-going.

Action: For information

Notes:
See 6.8.1. Table 6. Evaluation Ongoing.

3. Requests for protocol assistance with significant benefit question

3.1. Ongoing procedures

3.1.1. -

Treatment in haematopoietic stem cell transplantation

Action: For adoption

3.2. Finalised letters

3.2.1. -

Treatment of tuberculosis

Action: For information

Document tabled:
Final Advice Letter

Notes:
Final Advice Letter adopted at the October 2016 CHMP

3.3. New requests

3.3.1. -

Treatment of acromegaly

Action: For information

4. Review of orphan designation for orphan medicinal products for marketing authorisation

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

4.1.1. Chenodeoxycholic acid sigma-tau - chenodeoxycholic acid – EMA/OD/196/14, EU/3/14/1406, EMEA/H/C/004061

Sigma-tau Arzneimittel GmbH; Treatment of inborn errors of primary bile acid synthesis

Action: For information

Notes:
Status of the procedure at the CHMP: Opinion adopted in September 2016.

4.1.2. Cystadrops (mercaptamine) - EMA/OD/036/08, EU/3/08/578, EMEA/H/C/003769

Orphan Europe S.A.R.L.; Treatment of cystinosis

Action: For discussion

Notes:
Status of the procedure at the CHMP: Opinion adopted in October 2016.

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

4.2.1. - trientine tetrahydrochloride – EMA/OD/001/15, EU/3/15/1471, EMEA/H/C/004005

GMP-Orphan SA; Treatment of Wilson's disease

Action: For discussion

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

4.2.2. - eryaspase – EMEA/OD/033/06, EU/3/06/409, EMEA/H/C/004055

ERYTECH Pharma S.A.; Treatment of acute lymphoblastic leukaemia

Action: For discussion

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

4.2.3. - dinutuximab beta – EMA/OD/112/12, EU/3/12/1062, EMEA/H/C/003918

APEIRON Biologics AG; Treatment of neuroblastoma

Action: For discussion

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

4.2.4. - aceneuramic acid – EMA/OD/126/11, EU/3/12/972, EMEA/H/C/004176

Ultragenyx UK Limited; Treatment of GNE myopathy

Action: For discussion

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

4.3. On-going procedures

Action: For information

4.4. Public Summary of Opinion

Action: For information

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

None

6. Organisational, regulatory and methodological matters

6.1. Mandate and organisation of the COMP

6.1.1. Strategic Review & Learning meetings

Report from COMP Strategy Review & Learning meetings, 17-18 October 2016, Rome, Italy

Action: For information

Document tabled:
Presentations

6.1.2. Protocol Assistance Working Group

Proposed meeting time on 4 November 2016 at time 08:00, room 2H (To be confirmed)

Document(s) tabled:
Pending

6.1.3. COMP Drafting Group

Proposed meeting time on 4 November 2016 at time 13:30, room 2H (To be confirmed)

6.1.4. Preclinical Models Working Group

None

6.1.5. Recommendations on eligibility to PRIME – report from CHMP

Action: For information

Document(s) tabled:
PRIME eligibility requests - list of adopted outcomes October 2016

6.2. Coordination with EMA Scientific Committees or CMDh-v

6.2.1. PDCO/COMP Working Group

Proposed meeting time on 3 November 2016 at 13:00 (Cancelled)

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

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

None

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

None

6.4. Cooperation within the EU regulatory network

6.4.1. The European Network of Centres for Pharmacoepidemiology & Pharmacovigilance (ENCePP)

COMP representative at ENCePP: renewal of Dinah Duarte for a second term

Action: For adoption

6.5. Cooperation with International Regulators

6.5.1. Food and Drug Administration (FDA)

Action: For information

Document tabled:
Draft Agenda October 11, 2016

Notes:
Monthly teleconference

6.5.2. Japanese Pharmaceuticals and Medical Devices Agency (PMDA)

None

6.5.3. The Therapeutic Goods Administration (TGA), Australia

None

6.5.4. Health Canada

None

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

None

6.7. COMP work plan

6.7.1. COMP Work Plan 2016

Action: For information

Document(s) tabled:
COMP Work Plan 2016
COMP Work plan tracking tool 2016

6.7.2. COMP Work Plan 2017

Action: For information

Document(s) tabled:
COMP draft Work Plan 2017

6.8. Planning and reporting

6.8.1. List of all applications submitted/expected and the COMP coordinatorship distribution of valid applications submitted in 2016

Action: For information

6.8.2. Overview of orphan marketing authorisations/applications

Action: For information

7. Any other business

None

8. 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
FAL: Final Advice Letter
OD: Orphan Designation
OMPD: Orphan Medicinal Product 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/