16 March 2016
EMA/COMP/145890/2016
Procedure Management and Committees Support Division

Committee for Orphan Medicinal Products (COMP)

Draft agenda for the meeting on 21-23 March 2016

Chair: Bruno Sepodes – Vice-Chair: Lesley Greene

21 March 2016, 09:00-18:30, room 2F

22 March 2016, 08:30-18:30, room 2F

23 March 2016, 08:30-12:30, 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).

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

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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 21-23 March 2016. See March 2016 COMP minutes (to be published post April 2016 COMP meeting).

1.2. Adoption of agenda

COMP agenda for 21-23 March 2016.

1.3. Adoption of the minutes

COMP minutes for 16-18 February 2016.

2. Applications for orphan medicinal product designation

2.1. For opinion

2.1.1. - EMA/OD/204/15

Treatment of pancreatic cancer

Action: For adoption, Oral explanation to be held on 21 March 2016 at time 09:30

Documents tabled:
Draft Summary report with response to LoQs

Notes:
There are currently 34 designations for this condition: EMEA/OD/055/02 G17(9) gastrin-Diphtheria Toxoid Conjugate, EMEA/OD/068/02 Rubitecan, EMEA/OD/009/05 Bovine bile extract, EMEA/OD/028/05 4-imino-1, 3-diazobicyclo-[3.1.0]-hexan-2-one, EMEA/OD/063/06 Paclitaxel (liposomal), EMEA/OD/026/06 Human telomerase reverse transcriptase peptide (611-626), EMEA/OD/103/06 Cisplatin (liposomal), EMEA/OD/111/07 Chimeric antibody to mesothelin, 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/302/14 Human reovirus type 3 Dearing strain, EMA/OD/034/15 Modified adenovirus serotype 5/35 containing a CMV promoter-driven transgene cassette with the human transgenes for a membrane-bound CD40 ligand (TMZ-CD40L) and full length 4-1BBL, EMA/OD/168/15 Live attenuated Listeria monocytogenes delta actA/delta inlB strain expressing human mesothelin, EMA/OD/169/15 Two allogenic irradiated pancreatic tumour cell lines

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/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

2.1.2. - EMA/OD/212/15

Treatment of acute respiratory distress syndrome

Action: For adoption, Oral explanation to be held on 21 March 2016 at time 14:30

Documents tabled:
Draft Summary report with response to LoQs

Notes:
There are currently 2 designations for this condition: EMEA/OD/099/06 Drotrecogin alfa, EMA/OD/110/14 Imatinib

2.1.3. - EMA/OD/233/15

Treatment of acute myeloid leukaemia

Action: For adoption, Oral explanation to be held on 21 March 2016 at time 17:00

Documents tabled:
Draft Summary report with response to LoQs

Notes:
There are currently 46 designations for this condition: EMEA/OD/022/00 Gemtuzumab ozogamicin, EMA/OD/044/10 Allogeneic T cells encoding an exogenous TK gene, EMEA/OD/028/04 Midostaurin, EMEA/OD/051/04 Homoharringtonine, 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/056/06 Antisense oligonucleotide 5'-d[P-Thio] (CCCTG CTCCC CCCTG GCTCC)-3' (see comments box for cenersen sodium), 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/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/258/14 Ulocuplumab, 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/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

Designations withdrawn: EMEA/OD/065/02 2-chloro-9-[2-deoxy-2-fluoro-ß-D-arabinofuranosyl]adenine, 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 Ig-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

2.1.4. - EMA/OD/235/15

Treatment of osteogenesis imperfecta

Action: For information

Documents tabled:
Withdrawal request of 10 March 2016

Notes:
There is currently 1 designation for this condition: EMA/OD/053/15 Human allogeneic bone-marrow-derived osteoblastic cells

2.1.5. - EMA/OD/217/15

Prevention of short bowel syndrome

Action: For information

Documents tabled:
Withdrawal request of 4 March 2016

2.1.6. - EMA/OD/236/15

Treatment of Smith-Magenis syndrome

Action: For adoption, Oral explanation to be held on 22 March 2016 at time 11:00

Documents tabled:
Draft Summary report with response to LoQs

Notes:
Designations withdrawn: EMA/OD/260/14 Tasimelteon

2.1.7. - EMA/OD/234/15

Prevention of cytomegalovirus disease in patients with impaired cell-mediated immunity deemed at risk

Action: For adoption, Oral explanation to be held on 22 March 2016 at time 15:30

Documents tabled:
Draft Summary report with response to LoQs

Notes:
There is currently 1 designation for this condition: EMA/OD/090/10 (S)-{8-fluoro-2-2[4-(3-methoxyphenyl)-1-piperazinyl]-3-[2-methoxy-5-(trifluoromethyl)-phenyl]-3,4-dihydro-4-quinazolinyl} acetic acid

2.1.8. - EMA/OD/215/15

Treatment of soft tissue sarcoma

Action: For adoption, Oral explanation to be held on 22 March 2016 at time 17:00

Documents tabled:
Draft Summary report with response to LoQs

Notes:
There are currently 8 designations for this condition: EMEA/OD/001/01 Ecteinascidin 743, EMA/OD/155/11 Yttrium (90Y)-DTPA-radiolabelled chimeric monoclonal antibody against frizzled homologue 10, EMEA/OD/042/06 Doxorubicin hydrochloride (liposomal), EMA/OD/110/11 (1-methyl-2-nitro-1H-imidazole-5-yl)methyl N,N'-bis(2-bromoethyl) diamidophosphate, EMA/OD/041/13 Genetically modified serotype 5/3 adenovirus coding for granulocyte macrophage colony-stimulating factor, EMA/OD/190/13 Doxorubicin(6-maleimidocaproyl)hydrazone, EMA/OD/266/14 Olaratumab, EMA/OD/184/15 (S)-N-(5-((R)-2-(2,5-difluorophenyl)pyrrolidin-1-yl)pyrazolo[1,5-a]pyrimidin-3-yl)-3-hydroxypyrrolidine-1-carboxamide hydrogen sulfate

Designations withdrawn: EMEA/OD/059/03 N-acetylsarcosyl-glycyl-L-valyl-D-alloisoleucyl-L-threonyl-L-norvalyl-L-isoleucyl-L-arginyl-L-prolyl-N-ethylamide, EMEA/OD/050/05 (1R, 2R, 4S)-4-{(2R)-2-[(3S,6R,7E,9R,10R,12R,14S,15E,17E,19E,21S,23S,26R, 27R,34aS)-9,27-dihydroxy-10,21-dimethoxy-6,8,12,14,20,26-hexamethyl-1,5,11,28,29-pentaoxo-1,4,5,6,9,10,11,12,13,14,21,22,23,24,25,26,27,28,29,31,32,33,34,34a-tetra-co, EMEA/OD/071/05 Brostallicin, EMEA/OD/083/06 Fenretinide, EMEA/OD/044/08 Palifosfamide, EMA/OD/141/10 Ombrabulin

2.2. For discussion / preparation for an opinion

2.2.1. - EMA/OD/252/15

Treatment of spinal and bulbar muscular atrophy/Kennedy disease

Action: For adoption

Documents tabled:
Draft Summary report

2.2.2. - EMA/OD/253/15

Treatment of acute myeloid leukaemia

Action: For adoption

Documents tabled:
Draft Summary report

Notes:
There are currently 46 designations for this condition: Please see 2.1.3.

2.2.3. - EMA/OD/146/15

Treatment of congenital coronary artery malformation

Action: For adoption

Documents tabled:
Draft Summary report

2.2.4. - EMA/OD/255/15

Treatment of Leber's congenital amaurosis

Action: For adoption

Documents tabled:
Draft Summary report

Notes:
There are currently 5 designations for this condition: EMA/OD/150/11 Adenovirus associated viral vector serotype 2 containing the human RPE65 gene, EMA/OD/182/13 Adeno-associated viral vector serotype 8 containing the human GUCY2D gene, EMA/OD/163/10 9-cis-Retinyl acetate, EMEA/OD/072/07 Adenovirus associated viral vector serotype 4 containing the human RPE65 gene, EMA/OD/129/15 Adenovirus associated viral vector serotype 5 containing the human RPE65 gene

Designation withdrawn: EMA/OD/309/14 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

2.2.5. - EMA/OD/256/15

Treatment of inclusion body myositis

Action: For adoption

Documents tabled:
Draft Summary report

Notes:
There is currently 1 designation for this condition: EMA/OD/046/12 recombinant human monoclonal antibody against activin receptor type IIB

2.2.6. - EMA/OD/218/15

Treatment of epidermolysis bullosa

Action: For adoption

Documents tabled:
Draft Summary report

Notes:
There are currently 10 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/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

Designation withdrawn: EMA/OD/172/10 Human dermal fibroblasts cultured on a bioresorbable polyglactin mesh

2.2.7. - EMA/OD/257/15

Treatment of systemic sclerosis

Action: For adoption

Documents tabled:
Draft Summary report

Notes:
There are currently 13 designations for this condition: EMEA/OD/032/01 Halofuginone hydrobromide, EMEA/OD/035/05 Peptide 144 TGF-beta1-inhibitor (TSLDASIIWAMMQN), EMEA/OD/079/08 Type I native bovine skin collagen, EMEA/OD/106/08 Treprostinil diethanolamine, EMA/OD/095/10 Paquinimod, EMA/OD/143/12 2-[4-Methoxy-3-(2-m-tolyl-ethoxy)-benzoylamino]-indan-2-carboxylic acid, EMA/OD/153/12 Terguride, EMA/OD/044/14 Riociguat, EMA/OD/129/14 1-(6-benzothiazolylsulfonyl)-5-chloro-1H-indole-2-butanoic acid, EMA/OD/148/14 Humanized IgG1 monoclonal antibody against human eotaxin-2, EMA/OD/225/14 Nitroglycerin, EMA/OD/296/14 Autologous adipose tissue-derived stromal vascular fraction cells, EMA/OD/105/15 2-(2-chlorophenyl)-4-[3-(dimethylamino)phenyl]-5-methyl-1H-pyrazolo[4,3-C]pyridine-3,6(2H,5H)-dione

Designations withdrawn: EMEA/OD/051/01 Human engineered monoclonal antibody specific for Transforming Growth Factor ß1, EMA/OD/163/11 Pomalidomide, EMA/OD/156/12 Terguride

2.2.8. - EMA/OD/248/15

Prevention of graft versus host disease

Action: For adoption

Documents tabled:
Draft Summary report

2.2.9. - EMA/OD/251/15

Treatment of multiple symmetric lipomatosis

Action: For adoption

Documents tabled:
Draft Summary report

2.2.10. - EMA/OD/246/15

Treatment of paroxysmal nocturnal hemoglobinuria

Action: For adoption

Documents tabled:
Draft Summary report

2.2.11. - EMA/OD/220/15

Treatment of peripheral T-cell lymphoma

Action: For adoption

Documents tabled:
Draft Summary report

Notes:
There are currently 8 designations for this condition: EMA/OD/047/12 Recombinant anti-CD3-bi-single-chain-Fv-diphtheria toxin fusion protein, EMA/OD/104/12 Alisertib, EMA/OD/103/12 Belinostat, EMEA/OD/056/05 (E)-(1S,4S,10S,21R)-7-[(Z)-ethylidene]-4,21-diisopropyl-2-oxa-12,13-dithia-5,8,20,23- tetraazabicyclo[8.7.6]tricos-16-ene-3,6,9,19,22-pentone, EMEA/OD/100/06 Pralatrexate, EMEA/OD/096/06 Zanolimumab, EMA/OD/112/10 Darinaparsin, EMA/OD/104/11 Mogamulizumab

2.2.12. - EMA/OD/219/15

Treatment of non-infectious uveitis

Action: For adoption

Documents tabled:
Draft Summary report

Notes:
There are currently 4 designations for this condition: EMA/OD/118/12 Voclosporin, EMA/OD/024/15 3-{[2,3,5,6-tetrafluoro-3'-(trifluoromethoxy)biphenyl-4-yl]carbamoyl}thiophene-2-carboxylic acid, EMA/OD/195/14 Autologous collagen type II-specific regulatory T cells, EMA/OD/320/14 Triamcinolone acetonide

2.2.13. - EMA/OD/239/15

Treatment of progressive supranuclear palsy

Action: For adoption

Documents tabled:
Draft Summary report

Notes:
There are currently 3 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

Designations withdrawn: EMEA/OD/074/09 4-benzyl-2-naphtalen-1-yl-1,2,4-thiadiazolidine-3,5-dione, EMEA/OD/129/09 Davunetide

2.2.14. - EMA/OD/258/15

Treatment of glioma

Action: For adoption

Documents tabled:
Draft Summary report

Notes:
There are currently 39 designations for this condition: EMEA/OD/004/02 Pseudomonas exotoxin (domains II/III)-Interleukin 13 chimeric protein, EMEA/OD/037/02 Iodine (131I) anti-nucleohistone H1 chimeric biotinylated monoclonal antibody, EMEA/OD/026/03 Herpes simplex virus lacking infected cell protein 34.5, EMEA/OD/055/03 Gimatecan, EMEA/OD/023/08 Topotecan hydrochloride (liposomal), EMEA/OD/034/08 Gadodiamide (liposomal), 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/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 lim+Instructions.d, 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

Designations withdrawn: EMEA/OD/067/01 Carmustine (solution for intratumoral injection), EMEA/OD/074/01 Human transferrin conjugated to mutant diptheria toxin, EMEA/OD/067/03 Cilengitide, EMA/OD/031/10 Glutathione-pegylated liposomal doxorubicin hydrochloride, 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/049/12 Humanised monoclonal antibody against epidermal growth factor receptor, EMA/OD/113/15 Dronabinol and cannabidiol

2.2.15. - EMA/OD/242/15

Treatment of beta thalassaemia intermedia and major

Action: For adoption

Documents tabled:
Draft Summary report

Notes:
There is currently 1 designation for this condition: EMA/OD/092/15 Synthetic hepcidin

2.2.16. - EMA/OD/249/15

Treatment of Angelman syndrome

Action: For adoption

Documents tabled:
Draft Summary report

2.2.17. - EMA/OD/241/15

Treatment of amyotrophic lateral sclerosis

Action: For adoption

Documents tabled:
Draft Summary report

Notes:
There are currently 14 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

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

2.2.18. - EMA/OD/254/15

Treatment of cutaneous T-cell lymphoma

Action: For adoption

Documents tabled:
Draft Summary report

Notes:
There are currently 11 designations for this condition: EMEA/OD/038/01 Denileukin diftitox, EMEA/OD/001/04 Human monoclonal antibody against CD4, EMEA/OD/001/05 (E)-(1S,4S,10S,21R)-7-[(Z)-ethylidene]-4,21-diisopropyl-2-oxa-12,13-dithia-5,8,20,23-tetraazabicyclo[8.7.6]tricos-16-ene-3,6,9,19,22-pentone, EMEA/OD/030/08 Miltefosine, EMEA/OD/135/09 Pralatrexate, EMA/OD/112/11 Chlormethine, EMA/OD/100/11 Brentuximab vedotin, EMA/OD/050/12 Naloxone hydrochloride dihydrate, EMA/OD/066/12 Recombinant anti-CD3-bi-single-chain-Fv-diphtheria toxin fusion protein, EMA/OD/084/14 Humanised IgG1 monoclonal antibody against human KIR3DL2, EMA/OD/033/15 Synthetic hypericin

Designations withdrawn: EMEA/OD/007/03 Adenovirus-Interferon gamma-coding DNA sequence, EMEA/OD/003/04 Suberolylanilide Hydroxamic acid, EMEA/OD/015/07 Panobinostat lactate

2.2.19. - EMA/OD/250/15

Treatment of pantothenate kinase-associated neurodegeneration (PKAN)

Action: For adoption

Documents tabled:
Draft Summary report

2.2.20. - EMA/OD/243/15

Treatment of malignant mesothelioma

Action: For adoption

Documents tabled:
Draft Summary report

Notes:
There are currently 8 designations for this condition: EMEA/OD/003/08 NGR-human Tumour Necrosis Factor, EMA/OD/063/12 Maytansinoid-conjugated human monoclonal antibody against mesothelin, EMA/OD/012/13 N-methyl-4-({4-[({3

methyl(methylsulfonyl)aminopyrazin-2-yl}methyl)amino]-5-(trifluoromethyl)pyrimidin-2-yl}amino)benzamide hydrochloride, EMA/OD/138/13 Autologous dendritic cells pulsed with allogeneic tumour cell lysate, EMA/OD/108/13 Amatuximab, EMA/OD/076/14 Pegylated recombinant arginine deiminase, EMA/OD/180/14 Genetically modified serotype 5/3 adenovirus coding for granulocyte macrophage colony-stimulating factor, EMA/OD/157/15 Live attenuated Listeria monocytogenes delta actA/delta inlB strain expressing human mesothelin

Designations withdrawn: EMEA/OD/022/01 Pemetrexed disodium, EMA/OD/028/10 Vorinostat, EMA/OD/168/14 5-[8-methyl-9-(1-methylethyl)-2-(4-morpholinyl)-9H-purin-6-yl]-2-pyrimidinamine

2.3. Amendment of an existing orphan drug designation

2.3.1. S)-ethyl 2-amino-3-(4-(2-amino-6((R)-1-(4-chloro-2-(3-methyl-1H-pyrazol-1-yl)phenyl)-2,2,2-trifluoroethoxy)pyrimidin-4-yl)phenyl)propanoate – EU/3/09/661

Ipsen Pharma – France; Treatment of carcinoid tumours

Action: For information; for potential appointment of the COMP coordinator

Document tabled:
Request for confirmation ODD amendment - 02Mar2016

Notes:
A letter was received from the sponsor requesting confirmation on the need to amend the orphan designation.

2.4. COMP opinions adopted via written procedure following previous meeting

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 21-23 March 2016 COMP meeting

2.7. Evaluation on-going

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

Action: For information

Notes:
Cross reference to other agenda point. 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 of hyperargininaemia

Action: For adoption

3.1.2. –

Treatment of argininosuccinic aciduria

Action: For adoption

3.1.3. -

Treatment of haemophilia A

Action: For adoption

3.2. Finalised letters

3.2.1. -

Diagnosis of gastro-entero-pancreatic neuroendocrine tumours

Action: For information

3.3. New requests

3.3.1. -

Treatment of primary sclerosing cholangitis

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. ALPROLIX - eftrenonacog alfa – EMEA/OD/012/07, EU/3/07/453, EMEA/H/C/004142

Biogen Idec Ltd; Treatment of haemophilia B

Action: For adoption, Oral explanation to be held on 21 March 2016 at time 11:00

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

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

4.1.2. Idelvion - albutrepenonacog alfa – EMEA/OD/117/09, EU/3/09/723, EMEA/H/C/003955

CSL Behring GmbH; Treatment of haemophilia B

Action: For adoption, Oral explanation to be held on 21 March 2016 at time 12:00

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

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

4.1.3. Empliciti - elotuzumab - EMA/OD/061/12, EU/3/12/1037, EMEA/H/C/003967

Bristol-Myers Squibb; Treatment of multiple myeloma

Action: For adoption, Oral explanation to be held on 22 March 2016 at time 14:30

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

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

4.1.4. Revlimid – Lenalidomide - Type II variation - EMA/OD/078/11, EU/3/11/924, EMEA/H/C/000717/II/0079

Celgene Europe Limited; Treatment of mantle cell lymphoma

Action: For information

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

4.1.5. COAGADEX - factor X - EMEA/OD/044/07, EU/3/07/471, EMEA/H/C/003855

BIO PRODUCTS LABORATORY; Treatment of hereditary factor X deficiency

COMP coordinator: Karri Penttilä and Josep Torrent-Farnell**Action**: For information

Notes:
Status of the procedure at the CHMP: Opinion adopted in January 2016.
The COMP opinion was adopted by written procedure following its February meeting.

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

4.2.1. - migalastat – EMEA/OD/105/05, EU/3/06/368, EMEA/H/C/004059

Amicus Therapeutics UK Ltd; Treatment of Fabry disease

Action: For discussion

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

4.2.2. - autologous cd34+ enriched cell fraction that contains cd34+ cells transduced with retroviral vector that encodes for the human ada cdna sequence EMEA/OD/053/05, EU/3/05/313, EMEA/H/C/003854

GlaxoSmithKline Trading Services; Treatment of severe combined immunodeficiency (SCID) due to adenosine deaminase (ADA) deficiency

Action: For discussion

Document(s) tabled:
Draft report on review of OMPD
D180 LoOI reports

4.2.3. – daratumumab - EMA/OD/038/13, EU/3/13/1153 , EMEA/H/C/004077

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

Action: For discussion

Document(s) tabled:
Draft report on review of OMPD
D120 LoQ report

4.2.4. Dropcys (CYSTIRANE) – mercaptamine – EMA/OD/106/14, EU/3/14/1341, EMEA/H/C/004038

Lucane Pharma; Treatment of cystinosis

Action: For discussion

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

4.2.5. - allogeneic T cells genetically modified to express suicide gene - EMEA/OD/041/03, EU/3/03/168, EMEA/H/C/002801

MolMed SpA; Adjunctive treatment in haematopoietic cell transplantation

Action: For information

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

4.3. On-going procedures

4.3.1. List of on-going procedures

Action: For information

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

5.1.1. –

6. Organisational, regulatory and methodological matters

6.1. Mandate and organisation of the COMP

6.1.1. Significant Benefit Working Group

Proposed meeting time on 22 March 2016 at time 13:30, room 2F.

6.1.2. Protocol Assistance Working Group

Proposed meeting time on 22 March 2016 at time 12:00, room 2F.

6.1.3. New internal guidance on management of confidentiality and declarations of interests for observers participating in EMA scientific meetings

The Agency has developed internal guidance on observers participating in EMA scientific meetings, focusing on management of confidentiality and declarations of interests.

Action: For information

Document(s) tabled:
Observers summary for Committees and WPs
Observers at EMA Meetings

6.2. Coordination with EMA Scientific Committees or CMDh-v

None

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

6.3.1. SAWP/COMP joint membership

Call of interest for a SAWP/COMP member

Action: For information

6.4. Cooperation within the EU regulatory network

6.4.1. European Commission

Revision of the Commission Regulation (EC) No 847/2000 of April 2000 laying down the provisions for implementation of the criteria for designation of a medicinal product as an orphan medicinal product and definitions of the concept 'similar medicinal product' and 'clinical superiority'

Action: For information

6.5. Cooperation with International Regulators

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

None

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

6.8.3. COMP meeting dates for 2017-2018

Action: For information

Document(s) tabled:
FINAL - Committee meeting dates - 2016 - 2018

7. Any other business

None