30 November 2015
EMA/COMP/760301/2015
Procedure Management and Committees Support Division

Committee for Orphan Medicinal Products (COMP)

Draft agenda for the meeting on 8-10 December 2015

Chair: Bruno Sepodes – Vice-Chair: Lesley Greene

08 December 2015, 09:00-19:00, room 2F

09 December 2015, 08:30-19:00, room 2F

10 December 2015, 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 8-10 December 2015. See December 2015 COMP minutes (to be published post January 2016 COMP meeting).

1.2. Adoption of agenda

COMP agenda for 8-10 December 2015.

1.3. Adoption of the minutes

COMP minutes for 10-12 November 2015.

2. Applications for orphan medicinal product designation

2.1. For opinion

2.1.1. - EMA/OD/148/15

Treatment of interstitial lung disease in children

Action: For adoption, Oral explanation to be held on 8 December 2015 at time 09:30

Documents tabled:
Draft Summary report with response to LoQs

2.1.2. - EMA/OD/160/15

Treatment of soft tissue sarcoma

Action: For adoption, Oral explanation to be held on 8 December 2015 at time 12:00

Documents tabled:
Draft Summary report with response to LoQs

Notes:
There are currently 7 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
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

2.1.3. - EMA/OD/116/15

Treatment of acute myeloid leukemia

Action: For adoption, Oral explanation to be held on 8 December 2015 at time 15:30

Documents tabled:
Draft Summary report with response to LoQs

Notes:
There have been 42 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/105/12 Liposomal daunorubicin, 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

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 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

2.1.4. - EMA/OD/166/15

Treatment of ovarian cancer

Action: For information

Documents tabled:
Withdrawal request of 20 November 2015

Notes:
Withdrawn
There are currently 30 designations for this condition: EMEA/OD/019/02 Oregovomab, EMEA/OD/080/03 Anti-epithelial cell adhesion molecule/anti-CD3 monoclonal antibody, EMEA/OD/044/03 Trabectedin, EMA/OD/014/10 Pyr-His-Trp-Ser-Tyr-D-Lys(doxorubicinylglutarate)-Leu-Arg-Pro-Gly-NH2, acetate salt, EMEA/OD/065/05 Imexon, EMEA/OD/061/06 Paclitaxel (micellar), EMEA/OD/063/07 Olaparib, 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/094/11 Vincaleukoblastin-23-oic acid, O4-deacetyl-2-[(2-mercaptoethoxy)carbonyl]hydrazide, disulfide with N-[4-[[(2-amino-3,4-dihydro-4-oxo-6-pteridinyl)methyl]amino]benzoyl]-L-gamma-glutamyl-L-alpha-aspartyl-L-arginyl-L-alpha-

aspartyl-L-alpha-aspartyl-L-cysteine, 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/114/12 Alisertib, 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/314/14 {2-amino-8-[4-(pyrrolidinylcarbonyl)phenyl]-(3H-benzo[f]azepin-4-yl)}-N,N-dipropylcarboxamide

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/071/09 Anti-EphA2 monoclonal antibody conjugated to maleimidocaproyl monomethylauristatin phenylalanine, 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

2.1.5. - EMA/OD/122/15

Treatment of post cardiac arrest syndrome

Action: For adoption, Oral explanation to be held on 9 December 2015 at time 15:30

Documents tabled:
Draft Summary report with response to LoQs

2.1.6. - EMA/OD/155/15

Treatment of myelodysplastic syndromes

Action: For information

Documents tabled:
Withdrawal request of 17 November 2015

Notes:
Withdrawn
There are currently 6 designations for this condition: EMEA/OD/059/01 Azacitidine, EMEA/OD/059/02 Decitabine, EMEA/OD/083/03 3-(4'aminoisoindoline-1'-one)-1-piperidine-2,6-dione , EMEA/OD/014/08 Sapacitabine, EMA/OD/161/11 (E)-2,4,6-trimethoxystyryl-3-carboxymethylamino-4-methoxybenzyl-sulfone sodium salt, EMA/OD/048/14 Recombinant fusion protein consisting of a modified form of the extracellular domain of human Activin Receptor IIB linked to the human IgG1 Fc domain

Designations withdrawn: EMEA/OD/047/00 Arsenic trioxide, EMEA/OD/117/08 Lintuzumab, EMEA/OD/033/09 Allogeneic ex vivo expanded umbilical cord blood cells

2.1.7. - EMA/OD/123/15

Treatment of arginase deficiency

Action: For adoption, Oral explanation to be held on 9 December 2015 at time 12:00

Documents tabled:
Draft Summary report with response to LoQs

Notes:
Designation withdrawn: EMA/OD/231/14 Sodium benzoate

2.1.8. - EMA/OD/124/15

Treatment of argininosuccinate lyase deficiency

Action: For adoption, Oral explanation to be held on 9 December 2015 at time 12:00

Documents tabled:
Draft Summary report with response to LoQs

Notes:
Designation withdrawn: EMA/OD/230/14 sodium benzoate, EMA/OD/229/14 Sodium benzoate

2.1.9. - EMA/OD/152/15

Treatment of pulmonary arterial hypertension

Action: For adoption, Oral explanation to be held on 9 December 2015 at time 14:30

Documents tabled:
Draft Summary report with response to LoQs

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

2.1.10. - EMA/OD/164/15

Treatment of non-infectious uveitis

Action: For adoption, Oral explanation to be held on 8 December at 17:00

Documents tabled:
Draft Summary report with response to LoQs

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

2.1.11. - EMA/OD/168/15

Treatment of pancreatic cancer

Action: For adoption, Oral explanation to be held on 9 December 2015 at time 17:00

Documents tabled:
Draft Summary report with response to LoQs

Notes:
There are currently 32 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

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

2.1.12. - EMA/OD/169/15

Treatment of pancreatic cancer

Action: For adoption, Oral explanation to be held on 9 December 2015 at time 17:00

Documents tabled:
Draft Summary report with response to LoQs

Notes:
There are currently 32 designations for this condition: Please see 2.1.11.

2.2. For discussion / preparation for an opinion

2.2.1. - EMA/OD/184/15

Treatment of soft tissue sarcoma

Action: For adoption

Documents tabled:
Draft Summary report

Notes:
There are currently 7 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

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

2.2.2. - EMA/OD/180/15

Treatment of acute promyelocytic leukaemia

Action: For adoption

Documents tabled:
Draft Summary report

Notes:
Designations withdrawn: EMEA/OD/031/00 Arsenic trioxide, EMEA/OD/002/09 Tamibarotene

2.2.3. - EMA/OD/178/15

Treatment of congenital alpha-1 antitrypsin deficiency

Action: For adoption

Documents tabled:

Draft Summary report

Notes:
There are currently 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)

2.2.4. - EMA/OD/132/15

Treatment of Burkitt lymphoma

Action: For adoption

Documents tabled:
Draft Summary report

Notes:
Designations withdrawn: EMA/OD/075/12 Sertraline

2.2.5. - EMA/OD/191/15

Treatment of acute radiation syndrome

Action: For adoption

Documents tabled:
Draft Summary report

Notes:
There is currently 1 designation for this condition: EMA/OD/060/15 Fibrinogen-coated albumin spheres

2.2.6. - EMA/OD/188/15

Treatment of dystrophic epidermolysis bullosa

Action: For adoption

Documents tabled:
Draft Summary report

Notes:
There is currently 1 designation for this condition: EMEA/OD/099/08 Skin equivalent graft genetically corrected with a COL7A1-encoding SIN retroviral vector

2.2.7. - EMA/OD/182/15

Treatment of retinal detachment

Action: For adoption

Documents tabled:
Draft Summary report

2.2.8. - EMA/OD/177/15

Treatment of autoimmune haemolytic anaemia

Action: For adoption

Documents tabled:
Draft Summary report

2.2.9. - EMA/OD/190/15

Treatment of globoid cell leukodystrophy (Krabbe disease)

Action: For adoption

Documents tabled:
Draft Summary report

Notes:
There is currently 1 designation for this condition: EMA/OD/042/11 Recombinant human galactocerebrosidase

2.2.10. - EMA/OD/161/15

Treatment of anal cancer

Action: For adoption

Documents tabled:
Draft Summary report

2.2.11. - EMA/OD/183/15

Treatment of gastric cancer

Action: For adoption

Documents tabled:
Draft Summary report

Notes:
There are currently 6 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/030/12 Ramucirumab, EMA/OD/012/14 Rilotumumab

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

2.2.12. - EMA/OD/121/15

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

2.2.13. - EMA/OD/186/15

Prevention of necrotising enterocolitis

Action: For adoption

Documents tabled:
Draft Summary report

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

2.2.14. - EMA/OD/189/15

Treatment of pantothenate kinase associated neurodegeneration

Action: For adoption

Documents tabled:
Draft Summary report

Notes:
Designations withdrawn: EMA/OD/154/11 Deferiprone

2.2.15. - EMA/OD/104/15

Treatment of C3 glomerulopathy

Action: For adoption

Documents tabled:
Draft Summary report

2.2.16. - EMA/OD/185/15

Treatment of partial deep dermal and full thickness burns

Action: For adoption

Documents tabled:
Draft Summary report

Notes:
There has been 1 designation for this condition: EMEA/OD/012/02 Purified bromelain

2.3. Revision of the COMP opinions

None

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 8-10 December 2015 COMP meeting

2.7. Evaluation on-going

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

Action: For information

Notes:
Cross reference to other agenda point. See 5.8.1. Table 6. Evaluation Ongoing.

3. Requests for protocol assistance with significant benefit question

3.1. Ongoing procedures

3.1.1. -

Treatment of ovarian cancer

Action: For adoption

3.1.2. -

Treatment of amyotrophic lateral sclerosis

Action: For adoption

3.1.3. -

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

Action: For discussion

3.1.4. -

Treatment of Niemann-Pick disease, type C

Action: For discussion

3.1.5. -

Treatment of cytomegalovirus disease in patients with impaired cell mediated immunity

Action: For discussion

3.1.6. -

Treatment of advanced ovarian cancer

Action: For discussion

3.1.7. -

Treatment of acute myeloid leukaemia

Action: For discussion

3.2. Finalised letters

3.2.1. -

Treatment of glycogen storage disease type II (Pompe's disease)

Action: For information

3.2.2. -

Treatment of growth hormone deficiency

Action: For information

3.2.3. -

Treatment of Prader-Willi syndrome

Action: For information

3.3. New requests

None

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. Wakix - 1-{3-[3-(4-chlorophenyl)propoxy]propyl}piperidine, hydrochloride - EMEA/OD/087/06, EU/3/07/459, EMEA/H/C/002616

Bioprojet; Treatment of narcolepsy

Action: For discussion, Oral explanation to be held on 8 December 2015 at time 11:00.

Document tabled:
Draft report on review of OMPD
CHMP AR

Notes:
Status of the procedure at the CHMP: CHMP opinion adopted in November 2015.

4.1.2. Spectrila - asparaginase – EMA/OD/063/04, EU/3/04/258, EMEA/H/C/002661

Medac Gesellschaft fuer klinische Spezialpraeparate mbH; Treatment of acute lymphoblastic leukaemia

Action: For discussion, Oral explanation to be held on 8 December at time 14:30.

Document tabled:
Draft report on review of OMPD
CHMP AR

Notes:
Status of the procedure at the CHMP: CHMP opinion adopted in November 2015.

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

4.2.1. – 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

Notes:
Status of the procedure at the CHMP: Oral explanation in November 2015.

4.2.2. - 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

Notes:
Status of the procedure at the CHMP: D120 LoQ adopted in October 2015.

4.2.3. - dexamethasone acetate – EMEA/OD/133/09, EU/3/10/745, EMEA/H/C/004071

LABORATOIRES CTRS; Treatment of multiple myeloma

Action: For discussion

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

Notes:
Status of the procedure at the CHMP: 2nd LoOI adopted in November 2015.

4.2.4. - 3-[5-(2-fluoro-phenyl)-[1,2,4]oxadiazole-3-yl]-benzoic acid – EMEA/OD/107/04, EU/3/05/277, EMEA/H/C/002720/II/0012

PTC Therapeutics International Limited; Treatment of cystic fibrosis

Action: For discussion

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. Organisational, regulatory and methodological matters

5.1. Mandate and organisation of the COMP

5.1.1. Strategic Review & Learning meetings

During the Dutch EU presidency, the Medicines Evaluation Board will organise a strategic and learning meeting for the CHMP and the COMP in Utrecht, The Netherlands.

The meeting will be held on 30th May 2015 to 1st June 2015 including a joint CHMP/COMP working session.

Action: For information

5.2. Coordination with EMA Scientific Committees or CMDh-v

None

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

5.3.1. Significant Benefit Working Group

Proposed meeting time on 10 December 2015 from 11:30-12:30, room 2F

Action: For information

5.3.2. Preclinical Model Working Group

Proposed meeting time on 9 December 2015 from 18:00-19:00, room 2F

Action: For information

5.4. Cooperation within the EU regulatory network

5.4.1. European Commission

Report on the Commission Expert Group on Rare Diseases meeting held on 12-13 November 2015

Action: For information

Note: To be presented by Bruno Sepodes

Document(s) tabled:
Draft agenda - EC Expert Group on Rare Diseases 12-13 November meeting
Draft minutes - 5th EC Expert Group on Rare Diseases meeting

5.4.2. European Commission

Public consultation on the Commission Notice on the application of Articles 3,5 and 7 of Regulation (EC) NO 141/2000 on Orphan Medicinal Products

Action: For discussion

Document(s) tabled:
Comments on the public consultation on the Notice of Orphan Medicinal Products

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

Report on the ENCePP Plenary meeting held on 24 November 2015

Action: For information

Note: To be presented by Dinah Duarte

5.5. Cooperation with International Regulators

None

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

None

5.7. COMP work plan

5.7.1. Draft COMP Work Plan 2016

Action: For adoption

Document tabled:
Draft COMP Work Plan 2016

5.8. Planning and reporting

5.8.1. List of all applications submitted/expected and the COMP coordinatorship distribution of valid applications submitted in 2015

Action: For information

5.8.2. Overview of orphan marketing authorisations/applications

Action: For information

6. Any other business

None