9 June 2016
EMA/COMP/356295/2016
Procedure Management and Committees Support Division

Committee for Orphan Medicinal Products (COMP)

Draft agenda for the meeting on 14-16 June 2016

Chair: Bruno Sepodes – Vice-Chair: Lesley Greene

14 June 2016, 09:00-19:00, room 2F

15 June 2016, 08:30-19:00, room 2F

16 June 2016, 08:30-15:00, room 2F

Health and safety information

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

Disclaimers

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

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

Note on access to documents

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

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

Table of contents

8. Explanatory notes 25

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 14-16 June 2016. See June 2016 COMP minutes (to be published post July 2016 COMP meeting).

1.2. Adoption of agenda

COMP agenda for 14-16 June 2016.

1.3. Adoption of the minutes

COMP minutes for 17-19 May 2016.

2. Applications for orphan medicinal product designation

2.1. For opinion

2.1.1. - EMA/OD/049/16

Prevention of hereditary angioedema attacks

Action: For information

Documents tabled:
Withdrawal request of 30 May 2016

2.1.2. - EMA/OD/041/16

Treatment of hyperargininaemia

Action: For adoption, Oral explanation to be held on 14 June 2016 at time 15:00

Documents tabled:
Draft Summary report

Notes:
There are currently 4 designations for this condition: EMA/OD/005/10 glyceryl tri-(4-phenybutyrate), EMA/OD/106/10 Human heterologous liver cells (for infusion) , EMA/OD/060/13 Heterologous human adult liver-derived progenitor cells , EMA/OD/123/15 Sodium benzoate

2.1.3. - EMA/OD/043/16

Treatment of ovarian cancer

Action: For information

Documents tabled:

Withdrawal request of 26 May 2016

Notes:
Withdrawn
There are currently 31 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 , EMEA/OD/061/06 Paclitaxel (micellar) , 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 , EMEA/OD/065/05 Imexon , 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/014/10 Pyr-His-Trp-Ser-Tyr-D-Lys(doxorubicinylglutarate)-Leu-Arg-Pro-Gly-NH2, acetate salt , 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 … , 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/126/15 (5S,8S,10aR)-N-benzhydryl-5-((S)-2-(methylamino)propanamido)-3-(3-methylbutanoyl)-6-oxodecahydropyrrolo[1,2-a][1,5]diazocine-8-carboxamide

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

2.1.4. - EMA/OD/046/16

Treatment of idiopathic pulmonary fibrosis

Action: For adoption, Oral explanation to be held on 15 June 2016 at time 11:00

Documents tabled:

Draft Summary report

Notes:
There are currently 12 designations for this condition: EMEA/OD/033/04 Heparin-Sodium , EMEA/OD/052/04 Pirfenidone , EMEA/OD/054/07 Interferon gamma , EMEA/OD/104/09 Macitentan , EMA/OD/079/10 2-(2-chlorophenyl)-4-[3-(dimethylamino)phenyl]-5-methyl-1H-pyrazolo[4,3-C]pyridine-

3,6(2H,5H)-dione , EMA/OD/091/11 4-[[9-[(3S)-tetrahydro-3-furanyl]-8-[(2,4,6-trifluorophenyl)amino]-9H-purin-2-yl]amino]-trans-cyclohexanol , EMA/OD/048/12 Recombinant human pentraxin-2 , EMA/OD/111/12 Tralokinumab , EMA/OD/186/12 nintedanib , EMA/OD/051/14 Humanised anti-alpha v beta 6 monoclonal antibody , EMA/OD/130/14 1-(6-benzothiazolylsulfonyl)-5-chloro-1H-indole-2-butanoic acid , EMA/OD/072/15 3-pentylbenzeneacetic acid sodium salt

Designations withdrawn: EMEA/OD/002/05 Interferon gamma , EMEA/OD/075/04 Acetylcysteine , EMEA/OD/105/07 Recombinant human monoclonal antibody against transforming growth factor beta-1, 2 and 3 , EMEA/OD/027/08 Bosentan , EMA/OD/029/10 Ambrisentan

2.1.5. - EMA/OD/035/16

Treatment of fibromyalgia

Action: For adoption, Oral explanation to be held on 15 June 2016 at time 12:00

Documents tabled:
Draft Summary report

2.1.6. - EMA/OD/028/16

Treatment of retinitis pigmentosa

Action: For adoption, Oral explanation to be held on 14 June 2016 at time 11:00

Documents tabled:
Draft Summary report

Notes:
There are currently 15 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/067/13 Unoprostone isopropyl , 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

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)

2.1.7. - EMA/OD/042/16

Treatment of Clostridium difficile infection

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

Documents tabled:
Draft Summary report

2.1.8. - EMA/OD/048/16

Treatment of lymphangioleiomyomatosis

Action: For adoption, Oral explanation to be held on 15 June 2016 at time 17:00

Documents tabled:
Draft Summary report

2.1.9. - EMA/OD/045/16

Treatment of lymphangioleiomyomatosis

Action: For adoption, Oral explanation to be held on 15 June 2016 at time 18:00

Documents tabled:
Draft Summary report

2.1.10. - EMA/OD/029/16

Treatment of bullous pemphigoid

Action: For adoption

Documents tabled:
Draft Summary report

2.2. For discussion / preparation for an opinion

2.2.1. - EMA/OD/037/16

Treatment of soft tissue sarcoma

Action: For adoption

Documents tabled:
Draft Summary report

Notes:
There are currently 11 designations for this condition: EMEA/OD/001/01 Ecteinascidin 743, 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/155/11 Yttrium (90Y)-DTPA-radiolabelled chimeric monoclonal antibody against frizzled homologue 10 , 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/159/15 Glucopyranosyl lipid A stable emulsion and recombinant New York esophageal squamous cell carcinoma-1 protein , 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 , EMA/OD/215/15 Human/murine chimeric monoclonal antibody against endoglin , EMA/OD/238/15 Sindbis virus envelope pseudotyped lentiviral vector encoding New York esophageal squamous cell carcinoma-1 protein

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-cosahydro-3H-23,27-epoxypyrido[2,1-c][1,4]oxazacyclohentriacontin-3-yl]propyl}-2-methoxy-cyclohexyldimethyl-phosphinate, 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/066/16

Treatment of Huntington's disease

Action: For adoption

Documents tabled:
Draft Summary report

Notes:
There are currently 5 designations for this condition: EMEA/OD/021/00 Ethyl Eicosapentaenoate, EMEA/OD/011/05 4-[3-(methylsulfonyl)phenyl]-1-propylpiperidine x HCl , EMEA/OD/066/09 6-chloro-2,3,4,9-tetrahydro-1H-carbazole-1-carboxamide , EMEA/OD/095/09 Lithium citrate tetrahydrate (in reverse- micelle formulation) , EMA/OD/192/14 2'-O-methyl phosphorothioate RNA oligonucleotide, 5' m5CUGm5CUGm5CUGm5CUGm5CUGm5CUGm5CUG-3'

Designation withdrawn: EMEA/OD/061/08 2,3,4,5 tetrahydro-2,8-dimethyl-5-[2-(6-methyl-3-pyridinyl)ethyl]-1H-pyrido[4,3-b]indole dihydrochloride

2.2.3. - EMA/OD/079/16

Treatment of narcolepsy

Action: For adoption

Documents tabled:
Draft Summary report

Notes:
There are currently 3 designations for this condition: EMEA/OD/087/06 1-{3-[3-(4-chlorophenyl)propoxy]propyl}piperidine, hydrochloride , EMA/OD/254/14 Mazindol , EMA/OD/002/15 Mazindol

Designation withdrawn: EMEA/OD/051/02 Sodium oxybate

2.2.4. - EMA/OD/050/16

Treatment of Huntington's disease

Action: For adoption

Documents tabled:
Draft Summary report

Notes:
There are currently 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

2.2.5. - EMA/OD/080/16

Treatment of graft versus host disease

Action: For adoption

Documents tabled:
Draft Summary report

Notes:
There is currently 1 designation for this condition: EMA/OD/017/16 Rimiducid.

2.2.6. - EMA/OD/078/16

Treatment of idiopathic dilated cardiomyopathy

Action: For adoption

Documents tabled:
Draft Summary report

2.2.7. - EMA/OD/064/16

Treatment of soft tissue sarcoma

Action: For adoption

Documents tabled:

Draft Summary report

Notes:
There are currently 11 designations for this condition: Please see 2.2.1.

2.2.8. - EMA/OD/075/16

Treatment of extranodal NK/T cell lymphoma, nasal type

Action: For adoption

Documents tabled:
Draft Summary report

2.2.9. - EMA/OD/076/16

Treatment of post-transplantation lymphoproliferative disorders

Action: For adoption

Documents tabled:
Draft Summary report

Notes:
Designation withdrawn: EMEA/OD/052/02 Monoclonal antibody to human interleukin-6

2.2.10. - EMA/OD/069/16

Treatment of tracheal stenosis

Action: For adoption

Documents tabled:
Draft Summary report

2.2.11. - EMA/OD/070/16

Treatment of adenovirus infection

Action: For adoption

Documents tabled:
Draft Summary report

2.2.12. - EMA/OD/081/16

Treatment of amyotrophic lateral sclerosis

Action: For adoption

Documents tabled:
Draft Summary report

Notes:
There are currently 15 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/241/15 Recombinant human cerebral dopamine neurotrophic factor

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/051/16

Treatment of West Nile virus infection

Action: For adoption

Documents tabled:
Draft Summary report

2.2.14. - EMA/OD/071/16

Treatment of echinococcosis

Action: For adoption

Documents tabled:
Draft Summary report

2.2.15. - EMA/OD/072/16

Treatment of hepatocellular carcinoma

Action: For adoption

Documents tabled:
Draft Summary report

Notes:
There are currently 18 designations for this condition: EMEA/OD/015/02 Thymalfasin, EMEA/OD/087/04 Pegylated arginine deiminase , EMEA/OD/048/04 Doxorubicine polyisohexylcyanoacrylate nanoparticles , EMEA/OD/018/05 Nemorubicin hydrochloride , EMEA/OD/109/05 Sorafenib tosylate , EMEA/OD/070/09 NGR-human tumour necrosis factor , EMEA/OD/076/09 Vaccinia GM-CSF/TK-deactivated virus , EMA/OD/065/10 (S)-10-[(dimethylamino)methyl]-4-ethyl-9-hydroxy-4-O-[alpha-(2”, 4”, 5”, 7”-tetranitro-9”-fluorenylideneaminooxy)propionyl]-1H-pyrano[3’, 4’, 6’, 7’]indolizino[1,2-beta]-quinoline-3, 14-(4H, 12H)-dione, hydrochloride , EMA/OD/096/10 Doxorubicin hydrochloride (in heat-sensitive liposomes) , EMA/OD/170/10 Sulfonated monophosphorylated mannose oligosaccharide , EMA/OD/003/11 Peretinoin , EMA/OD/045/11 Resminostat ,

EMA/OD/159/12 4-[2-(6-methylpyridin-2-yl)-5,6-dihydro-4H-pyrrolo[1,2-b]pyrazol-3-yl]-quinoline-6-carboxamide monohydrate , EMA/OD/115/13 Tivantinib , EMA/OD/160/14 Diaspirin cross-linked haemoglobin , EMA/OD/287/14 Lenvatinib , EMA/OD/087/15 2-(2-phenylvinyl)-4-[4-methylpiperazin-1-yl)]-6-(5-methyl-2H-pyrazol-3-yl-amino)-pyrimidine L(+) tartrate salt , EMA/OD/118/15 2-chloro-N6-(3-iodobenzyl)adenosine-5'-N-methyluronamide

Designations withdrawn: EMEA/OD/013/01 Seocalcitol, EMEA/OD/026/02 Doxorubicin carbon/iron magnetically targeted microparticles , EMEA/OD/032/03 Nolatrexed , EMEA/OD/090/07 N-[4-(3-amino-1H-indazol-4 yl)phenyl]-N'-(2-fluoro-5-methylphenyl) urea , EMEA/OD/046/07 4-[3,5-bis(trimethylsilyl)benzamido] benzoic acid , EMA/OD/075/11 Brivanib alaninate , EMA/OD/031/12 Ramucirumab

2.2.16. - EMA/OD/040/16

Treatment of congenital hyperinsulinism

Action: For adoption

Documents tabled:
Draft Summary report

Notes:
There are currently 2 designations for this condition: EMA/OD/140/11 Glucagon , EMA/OD/128/14 Glucagon

2.2.17. - EMA/OD/077/16

Treatment of paroxysmal nocturnal haemoglobinuria

Action: For adoption

Documents tabled:
Draft Summary report

Notes:
There are currently 2 designations for this condition: EMEA/OD/042/03 Eculizumab , EMA/OD/098/14 S3,S13-cyclo(D-tyrolsyl-L-isoleucyl-L-cysteinyl-L-valyl-1-methyl-L-tryptophyl-L-glutaminyl-L-aspartyl-L-tryptophyl-N-methyl-L-glycyl-L-alanyl-L-histidyl-L-arginyl-L-cysteinyl-N-methyl-L-isoleucinamide)

Designation withdrawn: EMEA/OD/016/02 Myristolated-peptidyl-recombinant Human CD59

2.2.18. - EMA/OD/063/16

Treatment of pro-opiomelanocortin deficiency

Action: For adoption

Documents tabled:
Draft Summary report

2.2.19. - EMA/OD/054/16

Treatment of N-acetylglutamate synthase deficiency

Action: For adoption

Documents tabled:
Draft Summary report

Notes:
There is currently 1 designation for this condition: EMA/OD/061/13 Heterologous human adult liver-derived progenitor cells

2.2.20. - EMA/OD/055/16

Treatment of lysinuric protein intolerance

Action: For adoption

Documents tabled:
Draft Summary report

2.2.21. - EMA/OD/056/16

Treatment of ornithine translocase deficiency

Action: For adoption

Documents tabled:
Draft Summary report

Notes:
There is currently 1 designation for this condition: EMA/OD/063/13 Heterologous human adult liver-derived progenitor cells,

2.2.22. - EMA/OD/057/16

Treatment of carbamoylphosphate synthetase I deficiency

Action: For adoption

Documents tabled:
Draft Summary report

2.2.23. - EMA/OD/058/16

Treatment of citrullinaemia type 1

Action: For adoption

Documents tabled:
Draft Summary report

Notes:

There are currently 3 designations for this condition: EMA/OD/003/10 glyceryl tri-(4-phenybutyrate) , EMA/OD/105/10 Human heterologous liver cells (for infusion) , EMA/OD/058/13 Heterologous human adult liver-derived progenitor cells

Designation withdrawn: EMA/OD/096/11 Sodium phenylbutyrate

2.2.24. - EMA/OD/059/16

Treatment of argininosuccinic aciduria

Action: For adoption

Documents tabled:
Draft Summary report

Notes:
There are currently 4 designations for this condition: EMA/OD/004/10 glyceryl tri-(4-phenybutyrate) , EMA/OD/107/10 Human heterologous liver cells (for infusion) , EMA/OD/059/13 Heterologous human adult liver-derived progenitor cells , EMA/OD/124/15 Sodium benzoate

2.2.25. - EMA/OD/060/16

Treatment of argininaemia

Action: For adoption

Documents tabled:
Draft Summary report

2.2.26. - EMA/OD/053/16

Treatment of ornithine transcarbamylase deficiency

Action: For adoption

Documents tabled:
Draft Summary report

Notes:
There are currently 3 designations for this condition: EMEA/OD/101/07 Heterologous human adult liver derived stem cells, EMA/OD/026/11 Heterologous human adult liver-derived stem cells , EMA/OD/227/15 Adeno-associated viral vector serotype 8 encoding human ornithine transcarbamylase

Designation withdrawn: EMA/OD/097/11 Sodium phenylbutyrate

2.2.27. - EMA/OD/068/16

Treatment of partial deep dermal and full thickness burns

Action: For adoption

Documents tabled:
Draft Summary report

Notes:
There are currently 2 designations for this condition: EMEA/OD/012/02 Purified bromelain, EMA/OD/163/15 Bilayer, engineered, collagen hydrogel-based skin graft composed of autologous keratinocytes and fibroblasts

2.2.28. - EMA/OD/074/16

Treatment of McArdle disease

Action: For adoption

Documents tabled:
Draft Summary report

2.2.29. - EMA/OD/073/16

Treatment of McArdle disease

Action: For adoption

Documents tabled:
Draft Summary report

2.2.30. - EMA/OD/082/16

Treatment of familial partial lipodystrophy

Action: For adoption

Documents tabled:
Draft Summary report

Notes:
There is currently 1 designation for this condition: EMA/OD/033/12 Metreleptin

2.2.31. - EMA/OD/067/16

Treatment of glioma

Action: For adoption

Documents tabled:
Draft Summary report

Notes:
There are currently 41 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/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 limdinator_Applica, 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/222/15 Delta-9-tetrahydrocannabinol and cannabidiol from extracts of the Cannabis sativa L. plant

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 , 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

2.3. 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 14-16 June 2016 COMP meeting

2.7. Evaluation on-going

Thirty two 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 microscopic polyangiitis

Action: For adoption

3.1.2. -

Treatment of granulomatosis with polyangiitis

Action: For adoption

3.1.3. -

Treatment of acute myeloid leukaemia

Action: For adoption

3.1.4. -

Treatment of growth hormone deficiency

Action: For adoption

3.2. Finalised letters

3.2.1. -

Treatment of primary sclerosing cholangitis

Action: For information

3.2.2. -

Treatment of beta thalassaemia intermedia and major

Action: For information

3.3. New requests

3.3.1. -

Treatment of soft tissue sarcoma

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. 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

CHMP rapporteur: Pierre Demolis; CHMP co-rapporteur: Filip Josephson

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

Documents tabled:
Revised draft Summary report
Sponsor's grounds for appeal
CHMP assessment report

Notes:
Status of the procedure at the CHMP: Opinion adopted in January 2016.
Appeal of the COMP negative opinion adopted in February 2016.

4.1.2. NINLARO - ixazomib – EMEA/H/C/003844, EU/3/11/899, EMA/OD/048/11

Takeda Pharma A/S; Treatment of multiple myeloma

Action: For information

Notes:
Status of the procedure at the CHMP: Negative opinion in May 2016

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

4.2.1. - 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 adoption, Oral explanation to be held on 16 June 2016 at time 09:30

Documents tabled:
Draft report on review of OMPD
CHMP assessment report

4.2.2. - drisapersen – EMA/OD/121/12, EU/3/12/1077, EMEA/H/C/003846

BioMarin International Limited; Treatment of Duchenne muscular dystrophy

Action: For information

4.2.3. - amikacin –EMA/OD/024/06, EU/3/06/387, EMEA/H/C/003936

Insmed Limited;

a) Treatment of Pseudomonas aeruginosa lung infection in cystic fibrosis (EMA/OD/024/06, EU/3/06/387)

b) Treatment of nontuberculous mycobacterial lung disease (EMA/OD/191/13, EU/3/14/1259)

Action: For discussion

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

4.2.4. - parathyroid hormone – EMA/OD/102/13, EU/3/13/1210, EMEA/H/C/003861

NPS Pharma Holdings Limited; Treatment of hypoparathyroidism

Action: For discussion

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

4.2.5. - 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

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

4.2.6. - 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 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

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 the Strategic Review & Learning meeting in Utrecht (NL), 31 May-1 June 2016

Action: For information

Document tabled:
Supporting documents

Notes: To be presented by Bruno Sepodes

6.1.2. Protocol Assistance Working Group

Proposed meeting time on 14 June 2016 at time 18:00, room 2H

6.1.3. COMP Drafting Group

Proposed meeting time on 15 June 2016 at time 08:30, room 2G

6.1.4. Organisation of COMP November meeting 2016

Action: For discussion

6.2. Coordination with EMA Scientific Committees or CMDh-v

6.2.1. PDCO/COMP Working Group

Proposed meeting time on 15 June 2016 at time 13:00 by teleconference

6.2.2. Recommendations on eligibility to PRIME – report from CHMP

Action: For information

Document tabled:
6-2-2 PRIME eligibility requests - list of adopted outcomes May 2016

Notes: To be presented by Bruno Sepodes

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

None

6.4. Cooperation within the EU regulatory network

6.4.1. European Commission

None

6.5. Cooperation with International Regulators

6.5.1. Food and Drug Administration (FDA)

Action: For information

Notes:
Monthly teleconference

6.5.2. Japanese Pharmaceuticals and Medical Devices Agency (PMDA)

Action: For information

Notes:
Ad hoc basis meeting

6.5.3. The Therapeutic Goods Administration (TGA), Australia

Action: For information

Notes:
Ad hoc basis meeting

6.5.4. Health Canada

Action: For information

Notes:
Ad hoc basis meeting

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. Follow up on COMP Work Plan 2016

Action: For discussion

Document tabled:
COMP Work Plan 2016

Notes: To be presented by Bruno Sepodes

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

7.1.1. EMA Business Pipeline activity and Horizon scanning

Action: For information

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
OD: Orphan Designation
PA: Protocol Assistance
PDCO: Paediatric Committee
PRAC: Pharmacovigilance and Risk Assessment Committee
SA: Scientific Advice
SAWP: Scientific Advice Working Party

Orphan Designation (*section 2 Applications for orphan medicinal product designation*)

The orphan designation is the appellation given to certain medicinal products under development that are intended to diagnose, prevent or treat rare conditions when they meet a pre-defined set of criteria foreseen in the legislation. Medicinal products which get the orphan status benefit from several incentives (fee reductions for regulatory procedures (including protocol assistance), national incentives for research and development, 10-year market exclusivity) aiming at stimulating the development and availability of treatments for patients suffering from rare diseases.
Orphan Designations are granted by Decisions of the European Commission based on opinions from the COMP. Orphan designated medicinal products are entered in the Community Register of Orphan Medicinal Products.

Protocol Assistance (*section 3 Requests for protocol assistance with significant benefit question*)

The protocol assistance is the help provided by the Agency to the sponsor of an orphan medicinal product, on the conduct of the various tests and trials necessary to demonstrate the quality, safety and efficacy of the medicinal product in view of the submission of an application for marketing authorisation.

Sponsor

Any legal or physical person, established in the Community, seeking to obtain or having obtained the designation of a medicinal product as an orphan medicinal product.

Maintenance of Orphan Designation (*section 4 Review of orphan designation for orphan medicinal products for marketing authorisation*).

At the time of marketing authorisation, the COMP will check if all criteria for orphan designation are still met. The designated orphan medicinal product should be removed from the Community Register of Orphan Medicinal Products if it is established that the criteria laid down in the legislation are no longer met.

More detailed information on the above terms can be found on the EMA website: www.ema.europa.eu/