17 May 2016
EMA/COMP/292880/2016
Procedure Management and Committees Support Division

Committee for Orphan Medicinal Products (COMP)

Draft agenda for the meeting on 17-19 May 2016

Chair: Bruno Sepodes – Vice-Chair: Lesley Greene

17 May 2016, 09:00-19:00, room 2F

18 May 2016, 08:30-18:30, room 2F

19 May 2016, 08:30-12:00, room 2F

Health and safety information

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

Disclaimers

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

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

Note on access to documents

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

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1. Introduction

1.1. Welcome and declarations of interest of members and experts

Pre-meeting list of participants and restrictions in relation to declarations of interests applicable to the items of the agenda for the COMP plenary session to be held 17-19 May 2016. See May2016 COMP minutes (to be published post June 2016 COMP meeting).

1.2. Adoption of agenda

COMP agenda for 17-19 May 2016.

1.3. Adoption of the minutes

COMP minutes for 19-21 April 2016.

2. Applications for orphan medicinal product designation

2.1. For opinion

2.1.1. - EMA/OD/014/16

Treatment of neuromyelitis optica

Action: For adoption, Oral explanation to be held on 17 May 2016 at time 09:30

Documents tabled:
Draft Summary report

Notes:
There is currently 1 designation for this condition: EMA/OD/087/13 Eculizumab

2.1.2. - EMA/OD/022/16

Treatment of acute liver failure

Action: For adoption, Oral explanation to be held on 17 May 2016 at time 11:00

Documents tabled:
Draft Summary report

Notes:
There are currently 6 designations for this condition: EMEA/OD/037/05 Human heterologous liver cells (for infusion), EMEA/OD/085/08 Recombinant human hepatocarcinoma-intestine-pancreas / pancreatic associated protein, EMA/OD/030/11 Cardiotrophin-1, EMA/OD/105/11 Ornithine phenylacetate, EMA/OD/153/11 Heterologous human adult liver-derived stem cells, EMA/OD/032/13 Immortalised human C3A hepatoblastoma cells

2.1.3. - EMA/OD/004/16

Treatment of eosinophilic oesophagitis

Action: For adoption, Oral explanation to be held on 17 Month 2016 at time 12:00

Documents tabled:
Draft Summary report with response to LoQs

Notes:
There are currently 2 designations for this condition: EMA/OD/078/13 Budesonide, EMA/OD/118/13 Human monoclonal antibody against human interleukin 13

2.1.4. - EMA/OD/007/16

Treatment of neonatal sepsis

Action: For adoption, Oral explanation to be held on 17 May 2016 at time 14:30

Documents tabled:
Draft Summary report

2.1.5. - EMA/OD/001/16

Treatment of necrotising enterocolitis

Action: For adoption, Oral explanation to be held on 17 May 2016 at time 14:30

Documents tabled:
Draft Summary report

2.1.6. - EMA/OD/018/16

Treatment of beta thalassaemia intermedia and major

Action: For adoption, Oral explanation to be held on 17 May 2016 at time 15:30

Documents tabled:
Draft Summary report

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

2.1.7. - EMA/OD/019/16

Treatment of Fanconi anaemia

Action: For adoption, Oral explanation to be held on 17 May 2016 at time 15:30

Documents tabled:
Draft Summary report

2.1.8. - EMA/OD/020/16

Treatment of severe combined immunodeficiency

Action: For adoption, Oral explanation to be held on 17 May 2016 at time 15:30

Documents tabled:
Draft Summary report

Notes:
There is currently 1 designation for this condition: EMEA/OD/060/00 Retroviral gamma c cDNA containing vector

2.1.9. - EMA/OD/021/16

Treatment of Wiskott-Aldrich syndrome

Action: For adoption, Oral explanation to be held on 17 May 2016 at time 15:30

Documents tabled:
Draft Summary report

Notes:
There are currently 2 designations for this condition: EMA/OD/014/12 Autologous CD34+ cells transfected with lentiviral vector containing the Wiskott-Aldrich syndrome protein gene, EMA/OD/104/13 Autologous CD34+ cells transduced with a lentiviral vector containing the human Wiskott-Aldrich syndrome gene

2.1.10. - EMA/OD/023/16

Treatment of acromegaly

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

Documents tabled:
Draft Summary report

Notes:
There are currently 4 designations for this condition: EMEA/OD/010/09 Octreotide chloride (lipid depot solution), EMEA/OD/051/09 Pasireotide, EMA/OD/107/12 Cyclo(-gamma-aminobutyryl-L-phenylalanyl-L-tryptophanyl-D-tryptophanyl-L-lysyl-L-threonyl-L phenylalanyl-N-3-carboxypropyl)-glycine amide, acetate salt , EMA/OD/042/13 Octreotide acetate (oral use)

Designation withdrawn: EMA/OD/108/11 Recombinant protein consisting of modified human growth hormone releasing hormone and the translocation and endopeptidase domains of botulinum toxin serotype D

2.1.11. - EMA/OD/009/16

Treatment of glioma

Action: For adoption, Oral explanation to be held on 17 May 2016 at time 18:00

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

2.1.12. - EMA/OD/005/16

Treatment of acute respiratory distress syndrome

Action: For adoption, Oral explanation to be held on 19 May 2016 at time 11:00

Documents tabled:
Draft Summary report

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

2.1.13. - EMA/OD/008/16

Treatment of acute lymphoblastic leukaemia

Action: For adoption, Oral explanation to be held on 18 May 2016 at time 14:30

Documents tabled:
Draft Summary report

Notes:
There are currently 20 designations for this condition: EMEA/OD/046/01 2-chloro-9-[2-deoxy-2-fluoro-ß-D-arabinofuranosyl]adenine , EMEA/OD/070/06 Forodesine hydrochloride, EMEA/OD/015/05 Nelarabine, EMEA/OD/074/05 Dasatinib, EMEA/OD/033/06 L-asparaginase encapsulated in erythrocytes, EMEA/OD/065/07 Mercaptopurine (oral liquid), EMEA/OD/064/07 Methotrexate (oral liquid), EMEA/OD/002/08 Vincristine sulphate liposomes, EMEA/OD/032/08 Pegylated L-asparaginase, EMEA/OD/114/08 Mercaptopurine (oral suspension), EMA/OD/097/10 Recombinant human histone H1.3 and recombinant human N-bis-met-histone H1.3, EMEA/OD/029/09 Blinatumomab, EMEA/OD/084/09 6-thioguanine (oral liquid), EMEA/OD/122/09 Benzamide, 3-(2-imidazo[1,2-b]pyridazin-3-ylethynyl)-4-methyl-N-[4-[(4-methyl-1-piperazinyl)methyl]-3-(trifluoromethyl)phenyl], EMA/OD/168/10 Pegylated recombinant Erwinia chrysanthemi L-asparaginase, EMA/OD/001/11 Allogeneic T cells encoding an exogenous thymidine kinase gene, EMA/OD/143/13 (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/120/14 Allogeneic CD34+ cells expanded ex-vivo with an aryl hydrocarbon receptor antagonist, EMA/OD/107/15 Autologous T cells transduced with retroviral vector encoding an anti-CD19 CD28/CD3-zeta chimeric antigen receptor, EMA/OD/090/15 Allogeneic umbilical cord blood cells treated ex vivo with 16,16-dimethyl prostaglandin E2

Designations withdrawn: EMEA/OD/022/03 Aplidine, EMEA/OD/063/04 L-Asparaginase, EMEA/OD/038/05 Imatinib mesilate, EMEA/OD/067/08 Allogeneic ex vivo expanded umbilical cord blood cells

2.1.14. - EMA/OD/010/16

Treatment of glioma

Action: For adoption, Oral explanation to be held on 18 May 2016 at time 15:30

Documents tabled:
Draft Summary report

Notes:
There are currently 41 designations for this condition: Please see 2.1.11.

2.1.15. - EMA/OD/016/16

Treatment of diffuse large B-cell lymphoma

Action: For adoption

Documents tabled:
Draft Summary report

Notes:
There are currently 7 designations for this condition: EMEA/OD/091/08 Recombinant hisitidine-tagged idiotype immunoglobulin Fab fragment of clonal B-cell receptors, EMA/OD/160/10 Lenalidomide, EMA/OD/116/13 Ibrutinib, EMA/OD/092/14 obinutuzumab, EMA/OD/215/14 Humanised Fc engineered monoclonal antibody against CD19, EMA/OD/005/15 Humanised anti-CD37 monoclonal antibody conjugated to maytansinoid DM1, EMA/OD/084/15 2'-deoxyguanosylyl-(3',5'-phosphoryl)-2'-deoxythymidylyl-(3',5'-phosphoryl)- 2'-deoxyguanosylyl-(3',5'-phosphoryl)-2'-deoxycytidylyl-(3',5'-phosphoryl)-2'-deoxycytidylyl -(3',5'-phosphoryl)-2'-deoxycytidylyl-(3',5'-phosphoryl)-2'-deoxyguanosdinator-glycine amide, acetate salt ,

Designation withdrawn: EMEA/OD/126/09 Pixantrone dimaleate

2.2. For discussion / preparation for an opinion

2.2.1. - EMA/OD/046/16

Treatment of idiopathic pulmonary fibrosis

Action: For adoption

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

2.2.2. - EMA/OD/034/16

Treatment of fragile X syndrome

Action: For adoption

Documents tabled:
Draft Summary report

Notes:
There are currently 6 designations for this condition: EMA/OD/144/10 R-baclofen, EMA/OD/059/12 Mavoglurant, EMA/OD/105/14 (3S)-(+)-(5-chloro-2-methoxyphenyl)-1,3-dihydro-3-fluoro-6-(trifluoromethyl)-2H-indol-2-one , EMA/OD/137/14 Acamprosate calcium, EMA/OD/253/14 Tideglusib, EMA/OD/055/15 Glycyl-L-2-methylprolyl-L-glutamic acid

2.2.3. - EMA/OD/028/16

Treatment of retinitis pigmentosa

Action: For adoption

Documents tabled:
Draft Summary report

Notes:
There are currently 17 designations for this condition: EMEA/OD/057/06 4,7,10,13,16,19-Docosahexaenoic acid, EMEA/OD/043/07 Adenovirus associated viral vector serotype 4 containing the human RPE65 gene, EMEA/OD/087/08 Recombinant human proinsulin, EMA/OD/162/10 9-cis-Retinyl acetate, EMA/OD/159/11 Encapsulated human retinal pigment epithelial cell line transfected with plasmid vector expressing human ciliary neurotrophic factor, EMA/OD/006/12 Recombinant human methionine proinsulin, EMA/OD/021/12 17-(Dimethylaminoethylamino)-17-demethoxygeldanamycin (after administration of adeno-associated viral vector encoding an inducible short hairpin RNA targeting claudin-5), EMA/OD/135/12 Adeno-associated viral vector encoding an inducible short hairpin RNA targeting claudin-5 (prior to administration of 17-dimethylaminoethylamino-17-demethocygeldanamycin), EMA/OD/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

2.2.4. - EMA/OD/043/16

Treatment of ovarian cancer

Action: For adoption

Documents tabled:
Draft Summary report

Notes:
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

2.2.5. - EMA/OD/049/16

Prevention of hereditary angioedema attacks

Action: For adoption

Documents tabled:
Draft Summary report

2.2.6. - EMA/OD/032/16

Treatment of creatine deficiency syndromes

Action: For adoption

Documents tabled:
Draft Summary report

2.2.7. - EMA/OD/012/16

Treatment of periodic paralysis

Action: For adoption

Documents tabled:
Draft Summary report

Notes:
There is currently 1 designation for this condition: EMA/OD/199/15 Diclofenamide

2.2.8. - EMA/OD/029/16

Treatment of bullous pemphigoid

Action: For adoption

Documents tabled:
Draft Summary report

2.2.9. - EMA/OD/047/16

Treatment of Crigler-Najjar syndrome

Action: For adoption

Documents tabled:
Draft Summary report

Notes:
There are currently 3 designations for this condition: EMEA/OD/039/07 Heterologous human adult liver derived stem cells, EMA/OD/082/14 Adeno-associated viral vector

serotype 8 containing the human UGT1A1 gene, EMA/OD/122/14 Adeno-associated viral vector serotype 8 containing the human UGT1A1 gene

2.2.10. - EMA/OD/035/16

Treatment of fibromyalgia

Action: For adoption

Documents tabled:
Draft Summary report

2.2.11. - EMA/OD/048/16

Treatment of lymphangioleiomyomatosis

Action: For adoption

Documents tabled:
Draft Summary report

2.2.12. - EMA/OD/041/16

Treatment of hyperargininaemia

Action: For adoption

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

2.2.13. - EMA/OD/042/16

Treatment of Clostridium difficile infection

Action: For adoption

Documents tabled:
Draft Summary report

2.2.14. - EMA/OD/052/16

Treatment of osteogenesis imperfecta

Action: For adoption

Documents tabled:
Draft Summary report

Notes:

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

2.2.15. - EMA/OD/030/16

Treatment of Guillain-Barré syndrome

Action: For adoption

Documents tabled:
Draft Summary report

Notes:
Designation withdrawn: EMEA/OD/101/06 Fampridine

2.2.16. - EMA/OD/033/16

Treatment of Prader-Willi syndrome

Action: For adoption

Documents tabled:
Draft Summary report

Notes:
There are currently 3 designations for this condition: EMA/OD/119/11 Carbetocin, EMA/OD/023/14 Beloranib, EMA/OD/054/14 Oxytocin

2.2.17. - EMA/OD/045/16

Treatment of lymphangioleiomyomatosis

Action: For adoption

Documents tabled:
Draft Summary report

2.2.18. - EMA/OD/031/16

Treatment of hypoparathyroidism

Action: For adoption

Documents tabled:
Draft Summary report

Notes:
There is currently 1 designation for this condition: EMA/OD/102/13 Recombinant human parathyroid hormone

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 17-19 May 2016 COMP meeting

2.7. Evaluation on-going

Thirty one 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 primary sclerosing cholangitis

Action: For adoption

3.1.2. -

Treatment of beta thalassaemia intermedia and major

Action: For adoption

3.2. Finalised letters

None

3.3. New requests

3.3.1. -

Treatment of microscopic polyangiitis

Action: For information

3.3.2. -

Treatment of granulomatosis with polyangiitis

Action: For information

3.3.3. -

Treatment of acute myeloid leukaemia

Action: For information

3.3.4. -

Treatment of growth hormone deficiency

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. Gazyvaro – obinutuzumab - Type II variation - EMA/OD/013/15, EU/3/15/1504, EMEA/H/C/002799/II/0007

Roche Registration Limited; Treatment of follicular lymphoma

CHMP rapporteur: Sinan B. Sarac; CHMP co-rapporteur: Pierre Demolis

Action: For adoption

Documents tabled:
Draft report on review of OMPD

Notes:
Status of the procedure at the CHMP: Opinion adopted in April 2016
COMP list of issues adopted by written response.

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

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

Takeda Pharma A/S; Treatment of multiple myeloma

Action: For discussion

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

4.2.2. - sirolimus – EMA/OD/021/11, EU/3/11/898, EMEA/H/C/003978

Santen Oy; Treatment of chronic non-infectious uveitis

Action: For information

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. COMP Drafting Group

Proposed meeting time on 18 May 2016 at time 12:00, room 2G

6.1.2. Protocol Assistance Working Group

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

6.1.3. Preclinical Models Working Group

Proposed meeting time on 19 May 2016 at time 08:30, room 2H

6.1.4. Training of new COMP members

Proposed training time on 19 May at time 13:00 – 15:00, room 2F

6.2. Coordination with EMA Scientific Committees or CMDh-v

6.2.1. PDCO/COMP Working Group

Report from the last meeting in April 2016

Action: For information

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

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

COMP representative at the PCWP: renewal of Daniel O'Connor for a second term

Action: For adoption

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

COMP representative at the HCPWP: renewal of Kateřina Kopečková for a second term

Action: For adoption

6.4. Cooperation within the EU regulatory network

6.4.1. European Commission

Comments received during the 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:
Annex 1 - Notice on orphan medicinal products 12-04-2016

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

7. Any other business

7.1.1. COMP Prevalence Survey

Action: For information

Document(s) tabled:

7 Prevalence survey – presentation

7 Prevalence survey - summary_02-05-2016

7.1.2. EMA Business Pipeline activity and Horizon scanning

Action: For information