12 June 2017
EMA/COMP/311421/2017
Inspections, Human Medicines Pharmacovigilance and Committees

Committee for Orphan Medicinal Products (COMP)

Draft agenda for the meeting on 13-15 June 2017

Chair: Bruno Sepodes – Vice-Chair: Lesley Greene

13 June 2017, 09:00-18:30, room 2F

14 June 2017, 08:30-18:30, room 2F

15 June 2017, 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 13-15 June 2017. See June 2017 COMP minutes (to be published post July 2017 COMP meeting).

1.2. Adoption of agenda

COMP agenda for 13-15 June 2017.

1.3. Adoption of the minutes

COMP minutes for 10-12 May 2017.

2. Applications for orphan medicinal product designation

2.1. For opinion

2.1.1. - EMA/OD/325/16

Treatment of spinal cord injury

Action: For adoption, Oral explanation to be held on 13 June 2017 at 09:30

Document(s) tabled:
Draft Summary report with response to LoQs

Notes:
There have been 4 designations for this condition: EMEA/OD/082/07 3-methoxy-pregnenolone, EMEA/OD/059/08 Recombinant human monoclonal antibody to human Nogo-A protein of the IgG4/kappa class, EMEA/OD/042/08 Filgrastim, EMA/OD/119/13 synthetic 12 amino acids peptide designed after subcommissural organ-spondin

Designation withdrawn: EMEA/OD/041/08 Autologous urothelial and smooth muscle cells

2.1.2. - EMA/OD/030/17

Treatment of amyotrophic lateral sclerosis

Action: For adoption, Oral explanation to be held on 13 June 2017 at 12:00

Document(s) tabled:
Draft Summary report with response to LoQs

Notes:
There have been 20 designations for this condition: EMEA/OD/053/06 Arimoclomol, EMEA/OD/102/07 Filgrastim, EMEA/OD/096/08 (6R)-4,5,6,7-tetrahydro-N6-propyl-2,6-benzothiazole-diamine dihydrochloride monohydrate, EMEA/OD/108/09 Recombinant human vascular endothelial growth factor, EMA/OD/043/11 Smilagenin, EMA/OD/106/11 S[+] apomorphine, EMA/OD/138/11 6-ethynyl-1-(pentan-3-yl)-1H-imidazo[4,5-b]pyrazin-2(3H)-

one, EMA/OD/011/13 Autologous bone marrow-derived mesenchymal stromal cells secreting neurotrophic factors , EMA/OD/023/13 Sodium chlorite , EMA/OD/044/13 Allogeneic motor neuron progenitor cells derived from human embryonic stem cells , EMA/OD/184/14 Edaravone , EMA/OD/283/14 Enoxacin , EMA/OD/032/15 Edaravone , EMA/OD/051/15 Hydrocinnamate-[Orn-Pro-dCha-Trp-Arg]acetate , EMA/OD/011/16 H-Phe-Ser-Arg-Tyr-Ala-Arg-OH-acetate, EMA/OD/241/15 Recombinant human cerebral dopamine neurotrophic factor , EMA/OD/081/16 Masitinib mesilate, EMA/OD/120/16 Synthetic ribonucleic acid oligonucleotide directed against superoxide dismutase 1 messenger ribonucleic acid , EMA/OD/182/16 Ibudilast, EMA/OD/242/16 Tauroursodeoxycholic acid

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

2.1.3. - EMA/OD/025/17

Treatment of pachyonychia congenita

Action: For adoption, Oral explanation to be held on 13 June 2017 at 15:30

Document(s) tabled:
Draft Summary report with response to LoQs

Notes:
There has been 1 designation for this condition: EMA/OD/028/13 Synthetic double-stranded siRNA oligonucleotide directed against the keratin 6a N171K mutation

2.1.4. - EMA/OD/263/16

Treatment of neonatal abstinence syndrome

Action: For information

Document(s) tabled:
Withdrawal request of 26 May 2017

2.1.5. - EMA/OD/295/16

Treatment of invasive candidiasis

Action: For information

Document(s) tabled:
Withdrawal request of 19 May 2017

2.1.6. - EMA/OD/032/17

Treatment of myotonic disorders

Action: For adoption, Oral explanation to be held on 14 June 2017 at 17:00

Document(s) tabled:
Draft Summary report with response to LoQs

Notes:
There have been 2 designations for this condition: EMA/OD/069/13 Mexiletine hydrochloride, EMA/OD/074/14 mexiletine hydrochloride

2.1.7. - EMA/OD/023/17

Treatment of sudden sensorineural hearing loss

Action: For adoption, Oral explanation to be held on 14 June 2017 at 12:00

Document(s) tabled:
Draft Summary report with response to LoQs

Notes:
There have been 2 designations for this condition: EMA/OD/114/16 R-azasetron besylate , EMA/OD/189/16 Pioglitazone hydrochloride

2.1.8. - EMA/OD/024/17

Treatment of mastocytosis

Action: For adoption, Oral explanation to be held on 14 June 2017 at 14:30

Document(s) tabled:
Draft Summary report with response to LoQs

Notes:
There have been 4 designations for this condition: EMEA/OD/062/04 N-(methyl-diazacyclohexyl-methylbenzamide)-azaphenyl-aminothiopyrrole, EMA/OD/016/10 Midostaurin, EMA/OD/075/14 Recombinant human diamine oxidase , EMA/OD/079/13 Cladribine

2.1.9. - EMA/OD/033/17

Treatment of ischemic optic neuropathy

Action: For adoption, Oral explanation to be held on 14 June 2017 at 15:30

Document(s) tabled:
Draft Summary report with response to LoQs

2.1.10. - EMA/OD/029/17

Treatment of neuroblastoma

Action: For information

Document(s) tabled:
Withdrawal request of 30 May 2017

Notes:
There have been 10 designations for this condition: EMEA/OD/096/07 Iodine (131I) iobenguane, EMEA/OD/093/09 16-base single-stranded PNA oligonucleotide linked to a 7-aminoacid peptide, EMA/OD/126/10 Eflornithine, EMA/OD/002/11 Chimeric monoclonal antibody against GD2, EMA/OD/020/12 16 base single stranded peptide nucleic acid oligonucleotide - 7 aminoacids peptide , EMA/OD/112/12 Chimeric monoclonal antibody

against GD2 , EMA/OD/199/14 Chimeric monoclonal antibody specific to O-acetyl-GD2 antigen , EMA/OD/326/14 Sodium 2-hydroxylinoleate , EMA/OD/136/15 N-[5-(3,5-difluorobenzyl)-1H-indazol-3-yl]-4-(4 methylpiperazin-1-yl)-2-(tetrahydro-2H-pyran-4-ylamino)benzamide , EMA/OD/271/16 Iodine (131I) murine IgG1 monoclonal antibody against CD276

Designation withdrawn: EMEA/OD/013/09 Murine monoclonal antibody to GD2

2.2. For discussion / preparation for an opinion

2.2.1. - EMA/OD/037/17

Treatment of gastrointestinal stromal tumors

Action: For discussion/adoption

Document(s) tabled:
Draft Summary report

Notes:
Designation withdrawn: EMEA/OD/093/06 Nilotinib hydrochloride monohydrate

2.2.2. - EMA/OD/057/17

Treatment of glioma

Action: For discussion/adoption

Document(s) tabled:
Draft Summary report

Notes:
There have been 39 designations for this condition: EMEA/OD/026/03 Herpes simplex virus lacking infected cell protein 34.5, EMEA/OD/055/03 Gimatecan, EMEA/OD/050/04 Biotinylated anti-tenascin monoclonal antibody for use with 90-Yttrium, EMEA/OD/038/04 Anti epidermal growth factor receptor antibody h-R3, EMEA/OD/030/05 Oligonucleotide phosphorothioate (TAAACGTTATAACGTTATGACGTCAT), sodium salt, EMEA/OD/068/05 Enzastaurin hydrochloride, EMEA/OD/110/05 4-[131I] iodo-L-phenylalanine, EMEA/OD/081/06 Autologous dendritic cells pulsed with autologous tumour cell lysate, EMEA/OD/038/07 Iodine (131I) Chlorotoxin, EMEA/OD/004/08 Recombinant fusion protein of circulary-permuted IL-4 and pseudomonas exotoxin A, [IL-4(38-37)-PE38KDEL], EMEA/OD/023/08 Topotecan hydrochloride (liposomal), EMEA/OD/034/08 Gadodiamide (liposomal), EMEA/OD/104/08 Autologous tumour-derived gp96 heat shock protein-peptide complex, EMEA/OD/098/09 Recombinant fusion protein consisting of the extracellular portion of CD95 fused to the Fc part of a human IgG1 molecule, EMA/OD/086/10 7-beta-hydroxycholesteryl-3-beta-oleate, EMA/OD/092/12 IL-12-secreting dendritic cells, loaded with autologous tumour lysate , EMA/OD/077/11 L-cysteine, L-leucyl-L-alpha-glutamyl-L-alpha-glutamyl-L-lysyl-L-lysylglycyl-L-asparaginyl-L-tyrosyl-L-valyl-L-valyl-L-threonyl-L-alpha-aspartyl-L-histidyl-S-[1-[(4-carboxycyclohexyl)methyl]-2,5-dioxo-3-pyrrolidinyl]-complex with keyhole limpet haemocyanin, EMA/OD/050/11 2-hydroxyoleic acid, EMA/OD/157/11 Adenovirus-associated vector containing human Fas-c gene , EMA/OD/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/086/13 Autologous ex vivo expanded leukocytes treated with 5-aza-2'-deoxycytidine , EMA/OD/001/14 Autologous dendritic cells pulsed with RNA from glioma stem cells , EMA/OD/107/13 Allogeneic and autologous haptenised and irradiated cells and cell lysates derived from glioma , EMA/OD/174/13 Autologous dendritic cells pulsed with tumour antigen-derived synthetic peptides (MAGE-1, HER-2, AIM-2, TRP-2, gp-100, and interleukin-13 receptor alpha) , EMA/OD/111/14 Recombinant human bone morphogenetic protein 4 , EMA/OD/003/14 Paclitaxel-succinate- Arg-Arg-Leu-Ser-Tyr-Ser-Arg-Arg-Arg-Phe , EMA/OD/065/14 Humanised recombinant monoclonal antibody against epidermal growth factor receptor conjugated to maleimidocaproyl monomethylauristatin F , EMA/OD/132/14 Olaptesed pegol , EMA/OD/200/14 5,5'-(4-(trifluromethyl)benzylazanediyl)bis(methylene)diquinolin-8-ol, EMA/OD/159/14 Chloroquine, EMA/OD/176/14 Adenovirus serotype 5 containing partial E1A deletion and an integrin-binding domain , EMA/OD/251/14 Recombinant human glutamate oxaloacetate transaminase 1 , EMA/OD/206/15 N-(4-Methoxyphenyl)-N,2,6-trimethylfuro[2,3-d]pyrimidin-4-amine , EMA/OD/009/16 Eflornithine, EMA/OD/222/15 Delta-9-tetrahydrocannabinol and cannabidiol from extracts of the Cannabis sativa L. plant , EMA/OD/067/16 Zoledronic acid , EMA/OD/085/16 Temozolomide, EMA/OD/215/16 5-aminolevulinic acid

Designations withdrawn: EMEA/OD/004/02 Pseudomonas exotoxin (domains II/III)-Interleukin 13 chimeric protein, EMEA/OD/074/01 Human transferrin conjugated to mutant diptheria toxin, EMEA/OD/067/01 Carmustine (solution for intratumoral injection), EMEA/OD/050/06 Iodine (131I) anti-tenascin monoclonal antibody 81C6, EMEA/OD/037/02 Iodine (131I) anti-nucleohistone H1 chimeric biotinylated monoclonal antibody, EMEA/OD/067/03 Cilengitide, EMEA/OD/050/07 Doxorubicin hydrochloride (drug eluting beads), EMEA/OD/051/07 Irinotecan hydrochloride (drug eluting beads), 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/019/12 Doxorubicin (administered after synthetic double-stranded siRNA oligonucleotide directed against claudin-5 complexed with polyethyleneimine) , EMA/OD/136/12 Synthetic double-stranded siRNA oligonucleotide directed against Claudin-5 complexed with polyethyleneimine (prior to administration of doxorubicin) , EMA/OD/113/15 Dronabinol and cannabidiol

2.2.3. - EMA/OD/054/17

Treatment of spinal cord injury

Action: For discussion/adoption

Document(s) tabled:
Draft Summary report

Notes:
There have been 4 designations for this condition: EMEA/OD/082/07 3-methoxy-pregnenolone, EMEA/OD/059/08 Recombinant human monoclonal antibody to human Nogo-A protein of the IgG4/kappa class, EMEA/OD/042/08 Filgrastim, EMA/OD/119/13 synthetic 12 amino acids peptide designed after subcommissural organ-spondin

Designation withdrawn: EMEA/OD/041/08 Autologous urothelial and smooth muscle cells

2.2.4. - EMA/OD/050/17

Treatment of myelodysplastic syndromes

Action: For discussion/adoption

Document(s) tabled:
Draft Summary report

Notes:
There have been 7 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 , EMA/OD/272/16 Humanised IgG4 monoclonal antibody to the human toll-like receptor type 2

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

2.2.5. - EMA/OD/045/17

Treatment of diffuse large B-cell lymphoma

Action: For discussion/adoption

Document(s) tabled:
Draft Summary report

Notes:
There have been 11 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/016/16 3-(5-amino-2-methyl-4-oxoquinazolin-3(4H)-yl)piperidine-2,6-dione hydrochloride, EMA/OD/087/16 autologous T cells transduced with lentiviral vector containing a chimeric antigen receptor directed against CD19 , EMA/OD/162/16 Valproic acid , EMA/OD/122/16 Venetoclax, EMA/OD/229/16 5-(4,6-dimorpholino-1,3,5-triazin-2-yl)-4-(trifluoromethyl)pyridin-2-amine

Designations withdrawn: EMEA/OD/126/09 Pixantrone dimaleate, 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'-deoxyguanosylyl-(3',5'-phosphoryl)-2'-deoxycytidilyl-(3',5'-phosphoryl)-2'-deoxycytidylyl-(3',5'-phosphoryl)-2'-deoxycytidylyl-(3',5'-phosphoryl)-2'-deoxycytidylyl-(3',5'-phosphoryl)-2'-deoxythymidylyl-(3',5'-phosphoryl)-2'-deoxyadenosyl-(3',5'-phosphoryl)-2'-deoxycytidylyl-(3',5'-phosphoryl)-2'-deoxyguanosylyl-(3',5'-phosphoryl)-2'-deoxycytidylyl-(3',5'-phosphoryl)-2'-deoxyguanosylyl-(3',5'-phosphoryl)-2'-deoxycytidylyl-(3',5'-phosphoryl)-2'-deoxyadenosyl-(3',5'-phosphoryl)-2'-deoxycytidylyl-(3',5'-phosphoryl)-2'-deoxyguanosylyl-2'-deoxycytidylyl-(3',5'-phosphoryl)-2'-deoxyadenosyl-(3',5'-phosphoryl)-2'-deoxycytidine, sodium salt

2.2.6. - EMA/OD/058/17

Treatment of primary hyperoxaluria

Action: For discussion/adoption

Document(s) tabled:
Draft Summary report

Notes:
There has been 1 designation for this condition: EMEA/OD/095/05 Oxalobacter formigenes strain HC-1

2.2.7. - EMA/OD/053/17

Treatment of primary mitochondrial myopathy

Action: For discussion/adoption

Document(s) tabled:
Draft Summary report

2.2.8. - EMA/OD/044/17

Treatment of lung allograft dysfunction associated with lung transplantation

Action: For discussion/adoption

Document(s) tabled:
Draft Summary report

2.2.9. - EMA/OD/036/17

Treatment of sickle cell disease

Action: For discussion/adoption

Document(s) tabled:
Draft Summary report

Notes:
There have been 12 designations for this condition: EMEA/OD/017/05 Extract of Sorghum bicolour leaf, Pterocarpus osun stem, Piper guineense seed and Caryophylli flower, EMEA/OD/107/08 2,2-dimethylbutyric acid, sodium salt, EMEA/OD/075/09 Pegylated carboxyhaemoglobin, EMA/OD/016/12 Levoglutamide , EMA/OD/040/12 Human Erythrocytes encapsulating Inositol Hexaphosphate , EMA/OD/026/12 Humanised monoclonal antibody targeting P-selectin , EMA/OD/162/12 Poloxamer 188 , EMA/OD/084/13 (1R,3R,4R,5S)-3-O-[2-O-benzoyl-3-O-(sodium(2S)-3-cyclohexyl-propanoate-2-yl)-β-D-galactopyranosyl]-4-O-(α-L-fucopyranosyl)-5-orothylamido-cyclohexane-1-carboxylic acid (ethyl-2-amidyl-ethyloxy-2-acetyl-(8-amino-1,3,6-naphthalene-tris sodium sulfonate) amide, EMA/OD/184/13 Autologous CD34+ haematopoietic stem cells transduced with lentiviral vector encoding the human beta A-T87Q-globin gene , EMA/OD/210/14 Sevufparin sodium, EMA/OD/187/16 2-hydroxy-6-((2-(1-isopropyl-1H-pyrazol-5-yl)pyridin-3-yl) methoxy)benzaldehyde, EMA/OD/144/16 Synthetic human hepcidin

Designation withdrawn: EMA/OD/249/14 5-hydroxymethyl-2-furfural

2.2.10. - EMA/OD/056/17

Treatment of basal cell carcinoma Nevus syndrome (Gorlin syndrome)

Action: For discussion/adoption

Document(s) tabled:
Draft Summary report

Notes:
Designations withdrawn: EMEA/OD/048/09 N-[6-(cis-2,6-DimItraconazoleethylmorpholin-4-yl)pyridine-3-yl]-2-methyl-4'-(trifluoromethoxy)[1,1'-biphenyl]-3-carboxamide

2.2.11. - EMA/OD/003/17

Treatment of N-acetylglutamate synthase deficiency

Action: For discussion/adoption

Document(s) tabled:
Draft Summary report

Notes:
There has been 1 designation for this condition: EMA/OD/185/16 sodium benzoate

2.2.12. - EMA/OD/327/16

Treatment in cardiopulmonary by-pass

Action: For discussion/adoption

Document(s) tabled:
Draft Summary report

2.2.13. - EMA/OD/052/17

Treatment of hepatocellular carcinoma

Action: For discussion/adoption

Document(s) tabled:
Draft Summary report

Notes:
There have been 19 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 , EMA/OD/072/16 Mifamurtide

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

2.2.14. - EMA/OD/043/17

Treatment of mucopolysaccharidosis type VI (Maroteaux-Lamy syndrome)

Action: For discussion/adoption

Document(s) tabled:
Draft Summary report

Notes:
There has been 1 designation for this condition: EMA/OD/147/10 Adeno-associated viral vector containing the human ARSB gene

2.2.15. - EMA/OD/048/17

Treatment of anti-MAG neuropathy

Action: For discussion/adoption

Document(s) tabled:
Draft Summary report

2.2.16. - EMA/OD/049/17

Prevention of bronchopulmonary dysplasia

Action: For discussion/adoption

Document(s) tabled:
Draft Summary report

Notes:
There have been 5 designations for this condition: EMA/OD/161/13 Caffeine citrate , EMA/OD/018/14 Retinol , EMA/OD/172/13 Recombinant human surfactant protein D , EMA/OD/270/14 Recombinant human club cell 10 KDa protein , EMA/OD/010/15 Allogeneic ex-vivo-expanded human umbilical cord blood-derived mesenchymal stem cells

2.2.17. - EMA/OD/051/17

Prevention of Retinopathy of Prematurity

Action: For discussion/adoption

Document(s) tabled:
Draft Summary report

2.2.18. - EMA/OD/038/17

Treatment of hepatocellular carcinoma

Action: For discussion/adoption

Document(s) tabled:
Draft Summary report

Notes:
There have been 19 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 , EMA/OD/072/16 Mifamurtide

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

2.3. Revision of the COMP opinions

None

2.4. Amendment of existing orphan designations

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(s) tabled:
OMPD applications - appointment of coord. at the 13-15 June 2017 COMP meeting

2.7. Evaluation on-going

Twenty one application for orphan designation will not be discussed as evaluation is on-going.

Action: For information

Notes:
See 7.8.1. Table 6. Evaluation Ongoing.

3. Requests for protocol assistance with significant benefit question

3.1. Ongoing procedures

3.1.1. -

Treatment of myasthenia gravis

Action: For adoption

3.1.2. -

Treatment of mercury toxicity

Action: For adoption

3.1.3. -

Treatment of acute hepatic porphyria

Action: For discussion

3.1.4. -

Treatment of Prader-Willi syndrome

Action: For discussion

3.1.5. -

Treatment of plasminogen deficiency

Action: For adoption

3.1.6. -

Treatment of graft-versus-host disease

Action: For adoption

3.2. Finalised letters

3.2.1. -

Treatment of haemophilia A

Action: For information

3.2.2. -

Prevention of graft-versus-host disease

Action: For information

3.2.3. -

Treatment of soft tissue sarcoma

Action: For information

3.3. New requests

3.3.1. -

Treatment in solid organ transplantation

Action: For information

3.3.2. -

Treatment of haemophilia A

Action: For information

3.3.3. -

Treatment of metaphyseal chondrodysplasia, Schmid-type

Action: For information

4. Review of orphan designation for orphan medicinal products at time of initial marketing authorisation

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

None

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

4.2.1. - ciclosporin – EMEA/OD/106/05, EU/3/06/360, EMEA/H/C/004411

Santen Oy; Treatment of vernal keratoconjunctivitis

Action: For adoption, Oral explanation to be held on 13 June 2017 at time 14:30

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

4.2.2. - alpha-1-antitrypsin – EMEA/OD/054/04, EU/3/04/244, EMEA/H/C/003934

Kamada BioPharma Limited; Treatment of emphysema secondary to congenital alpha-1-antitrypsin deficiency

Action: For information

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

4.2.3. - midostaurin – EMEA/H/C/004095

Novartis Europharm Ltd;

a) Treatment of mastocytosis, EMA/OD/016/10, EU/3/10/765

b) Treatment of acute myeloid leukaemia, EMEA/OD/028/04, EU/3/04/214

Action: For discussion

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

4.2.4. - telotristat ethyl – EMEA/OD/047/09, EU/3/09/661, EMEA/H/C/003937

Ipsen Pharma; Treatment of carcinoid syndrome

Action: For discussion

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

4.2.5. - lutetium 177Lu dotatate – EMEA/OD/093/07, EU/3/07/523, EMEA/H/C/004123

Advanced Accelerator Applications; Treatment of gastro-entero-pancreatic neuroendocrine tumours

Action: For discussion

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

4.2.6. Adcetris - Brentuximab vedotin – Type II variation - EMA/OD/100/11, EU/3/11/939, EMEA/H/C/002455/II/0048

Takeda Pharma A/S - Denmark; Treatment of cutaneous T-cell lymphoma

CHMP rapporteur: Paula Boudewina van Hennik; CHMP co-rapporteur: Jan Mueller-Berghaus

Action: For discussion

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

4.2.7. Soliris - eculizumab – Type II variation - EMEA/OD/062/14, EU/3/14/1304, EMEA/H/C/000791/II/0090

Alexion Europe SAS - France; Treatment of myasthenia gravis

CHMP rapporteur: Jorge Camarero Jiménez

Action: For discussion

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

4.3. Appeal

None

4.4. On-going procedures

Action: For information

Document(s) tabled:
Review of orphan designation for OMP for MA - On-going procedures

4.5. Public Summary of Opinions

Action: For information

5. Review of orphan designation for authorised orphan medicinal products at time marketing authorisation extension

5.1. After adoption of CHMP opinion

None

5.2. Prior to adoption of CHMP opinion

5.2.1. Blincyto (blinatumomab) - Type II variation – EMEA/OD/029/09, EU/3/09/650, EMEA/H/C/003731/II/0011

Amgen Europe BV - The Netherlands; Treatment of acute lymphoblastic leukaemia

CHMP rapporteur: Alexandre Moreau; CHMP co-rapporteur: Daniela Melchiorri

Action: For discussion

Documents tabled:
Draft report on review of OMPD

5.2.2. Lynparza - Olaparib – EMEA/OD/063/07, EU/3/07/501, EMEA/H/C/003726/X/0016/G

AstraZeneca AB - Sweden; Treatment of ovarian cancer

CHMP rapporteur: Alexandre Moreau; CHMP co-rapporteur: Bart Van der Schueren

Action: For information

Documents tabled:
Draft report on review of OMPD

5.3. Appeal

None

5.4. On-going procedures

Action: For information

Document(s) tabled:
Review of orphan designation for OMP for MA extension - On-going procedures

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

None

7. Organisational, regulatory and methodological matters

7.1. Mandate and organisation of the COMP

7.1.1. Strategic Review & Learning meetings

None

7.1.2. Protocol Assistance Working Group

Proposed meeting time on 13 June 2017 at 13:00

Document(s) tabled:
PAWG draft agenda for June 2017 meeting
PAWG draft minutes for May 2017 meeting

7.1.3. Preclinical Models Working Group

Proposed meeting time on 15 June 2017 at 09:00

7.1.4. Conditions Steering Group

Proposed meeting time on 14 June 2017 at 18:00

7.2. Coordination with EMA Scientific Committees or CMDh-v

7.2.1. PDCO/COMP Working Group

Proposed meeting time on 14 June 2017 at 13:00 (TBC)

7.2.2. Recommendations on eligibility to PRIME – report from CHMP

Action: For information

Document(s) tabled:
PRIME eligibility requests - list of adopted outcomes May 2017

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

7.3.1. Working Party with Patients' and Consumers' Organisations (PCWP) and Healthcare Professionals' Organisations (HCPWP)

PCWP/HCPWP joint meeting – 27/28 June 2017

Action: For information

Document(s) tabled:
Draft Agenda of the PCWP/HCPWP joint meeting – 27/28 June (EMA/213892/2017)

7.4. Cooperation within the EU regulatory network

7.4.1. European Commission

Presentation on EC studies on pharmaceutical incentives

Action: For information

Notes:
To be presented by EC representative

7.5. Cooperation with International Regulators

7.5.1. Food and Drug Administration (FDA)

Action: For information

Document(s) tabled:
Draft Agenda of EMA/FDA teleconference on Orphan Medicines May 16, 2017

Notes:
Monthly teleconference

7.5.2. Japanese Pharmaceuticals and Medical Devices Agency (PMDA)

Action: For information

Notes:
Ad hoc basis meeting

7.5.3. The Therapeutic Goods Administration (TGA), Australia

Action: For information

Notes:
Ad hoc basis meeting

7.5.4. Health Canada

Action: For information

Notes:
Ad hoc basis meeting

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

None

7.7. COMP work plan

Action: For information

Document(s) tabled:
COMP Work Plan 2017

7.8. Planning and reporting

7.8.1. List of all applications submitted/expected and the COMP coordinatorship distribution of valid applications submitted in 2017

Action: For information

7.8.2. Overview of orphan marketing authorisations/applications

Action: For information

8. Any other business

8.1. Presentation "Bridging the regulator and the payer world, how far are we?"

Action: For information

8.2. EMA Business Pipeline activity and Horizon scanning

Action: For information

Document tabled:
Upcoming Q2/2017 Update of the Business Pipeline report for the human scientific committees

9. 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/