11 May 2017
EMA/COMP/246330/2017
Procedure Management and Committees Support Division

Committee for Orphan Medicinal Products (COMP)

Draft agenda for the meeting on 10-12 May 2017

Chair: Bruno Sepodes – Vice-Chair: Lesley Greene

10 May 2017, 09:00-19:00, room 2F

11 May 2017, 08:30-19:00, room 2F

12 May 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 10-12 May 2017. See May 2017 COMP minutes (to be published post June 2017 COMP meeting).

1.2. Adoption of agenda

COMP agenda for 10-12 May 2017.

1.3. Adoption of the minutes

COMP minutes for 10-11 April 2017.

2. Applications for orphan medicinal product designation

2.1. For opinion

2.1.1. - EMA/OD/005/17

Treatment of glioma

Action: For adoption, Oral explanation to be held on 10 May 2017 at 09:30

Documents tabled:
Draft Summary report with response to LoQs

Notes:
There have been 43 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/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/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/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

2.1.2. - EMA/OD/017/17

Treatment of cystic fibrosis

Action: For adoption, Oral explanation to be held on 10 May 2017 at 12:00

Documents tabled:
Draft Summary report with response to LoQs

Notes:
There have been 39 designations for this condition: EMEA/OD/032/00 L-Lysine-N-Acetyl-L-Cysteinate, EMEA/OD/011/03 Recombinant dog gastric lipase, EMEA/OD/038/02 Duramycin, EMEA/OD/039/04 Dexamethasone sodium phosphate encapsulated in human erythrocytes, EMEA/OD/053/04 Alpha-1 antitrypsin (inhalation use), EMEA/OD/107/04 3-[5-(2-fluoro-phenyl)-[1,2,4]oxadiazole-3-yl]-benzoic acid, EMEA/OD/062/05 Mannitolum,

EMEA/OD/001/06 Heparin sodium, EMEA/OD/037/09 Ciprofloxacin (liposomal), EMEA/OD/092/06 Ciprofloxacin (inhalation use), EMEA/OD/104/06 Alginate oligosaccharide (G-block) fragment, EMEA/OD/041/07 Alpha1-proteinase inhibitor (inhalation use), EMEA/OD/031/08 Avian polyclonal IgY antibody against Pseudomonas aeruginosa, EMEA/OD/010/08 N-(2,4-Di-tert-butyl-5-hydroxyphenyl)-1,4-dihydro-4-oxoquinoline-3-carboxamide, EMEA/OD/009/09 Hypothiocyanite / lactoferrin, EMA/OD/040/10 Nafamostat mesilate, EMA/OD/024/10 3-(6-(1-(2,2-difluorobenzo [d] [1,3] dioxol-5-yl)cyclopropanecarboxamido)-3-methylpyridin-2-yl)benzoic acid, EMA/OD/032/11 Sinapultide, dipalmitoylphosphatidylcholine palmitoyl-oleoyl phosphatidylglycerol, sodium salt and palmitic acid, EMA/OD/037/11 Multilamellar microvesicle comprising phosphatidylcholine, sphingomyelin, phosphatidylethanolamine, phosphatidylserine, phospatidylinositol and cholesterol, EMA/OD/046/11 Cysteamine, EMA/OD/058/12 Alpha-1 proteinase inhibitor (for inhalation use), EMA/OD/005/13 Recombinant human CXCL8 mutant, EMA/OD/017/13 4,6,4'-trymethylangelicin, EMA/OD/096/13 Antisense oligonucleotide targeting the F508delta mutation of CFTR, EMA/OD/095/13 Nitric oxide, EMA/OD/159/13 Cysteamine, EMA/OD/156/13 11-(4-Dimethylamino-3-hydroxy-6-methyl-tetrahydro-pyran-2-yloxy)-2-ethyl-3,4,10-trihydroxy-3,5,6,8,10,12,14-heptamethyl-1-oxa-6-aza-cyclopentadecane-13,15-dione, EMA/OD/036/14 Nitric oxide, EMA/OD/013/14 Plasmid DNA encoding the human cystic fibrosis transmembrane conductance regulator gene complexed with a non-viral, cationic lipid based gene transfer agent, EMA/OD/002/14 1-(2,2-difluoro-1,3-benzodioxol-5-yl)-N-{1-[(2R)-2,3-dihydroxypropyl]-6-fluoro-2-(1-hydroxy-2-methylpropan-2-yl)-1Hindol-5-yl}cyclopropanecarboxamide, EMA/OD/131/14 4-[[(1S,4S)-5-[[4-[4-(Oxazol-2-yl)phenoxy]phenyl]methyl]-2,5-diazabicyclo[2.2.1]hept-2-yl]methyl]benzoic acid, EMA/OD/018/15 2-(7-ethoxy-4-(3-fluorophenyl)-1-oxophthalazin-2(1H)-yl)-N-methyl-N-(2-methylbenzo[d]oxazol-6-yl)acetamide, EMA/OD/319/14 Nitric oxide, EMA/OD/068/15 Fixed-dose combination of fosfomycin disodium and tobramycin, EMA/OD/061/15 Recombinant human acid ceramidase, EMA/OD/013/16 Sodium nitrite and ethylenediaminetetraacetic acid, EMA/OD/156/16 1-(2,2-difluoro-2H-1,3-benzodioxol-5-yl)-N-{1-[(2R)-2,3-dihydroxypropyl]-6-fluoro-2-(1-hydroxy-2-methylpropan-2-yl)-1H-indol-5-yl}cyclopropane-1-carboxamide and ivacaftor, EMA/OD/100/16 (6aR, 10aR)-3-(1',1'-dimethylheptyl)- delta-8-tetrahydro-cannabinol-9-carboxylic acid, EMA/OD/303/16 Phosphoinositide 3-kinase gamma peptide

Designations withdrawn: EMEA/OD/009/02 Carbamic acid/[[4-[[3-[[4-[1-(4-hydroxyphenyl)-1-methyl-ethyl]phenoxy]methyl]phenyl]methoxy]-phenyl]iminomethyl]-,ethyl ester, EMEA/OD/064/00 8-cyclopentyl-1, 3-dipropylxanthine, EMEA/OD/018/03 Engineered protein inhibitor of human neutrophil elastase, EMEA/OD/075/02 Amiloride hydrochloride dihydrate, EMEA/OD/023/04 Recombinant human bile salt-stimulated lipase, EMEA/OD/054/05 Heparin sodium (inhalation use), EMEA/OD/072/05 Denufosol tetrasodium, EMEA/OD/118/05 Glutathione, EMEA/OD/024/08 Levofloxacin hemihydrate, EMA/OD/032/14 Lumacaftor/ivacaftor

2.1.3. - EMA/OD/324/16

Treatment of spinal cord Injury

Action: For adoption, Oral explanation to be held on 10 May 2017 at 17:00

Documents 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

2.1.4. - EMA/OD/002/17

Treatment of congenital hyperinsulinism

Action: For adoption, Oral explanation to be held on 10 May 2017 at time 18:00

Documents tabled:
Draft Summary report with response to LoQs

Notes:
There have been 3 designations for this condition: EMA/OD/140/11 Glucagon, EMA/OD/128/14 Glucagon, EMA/OD/040/16 Recombinant human monoclonal antibody to insulin receptor

2.1.5. - EMA/OD/018/17

Treatment of haemophilia B

Action: For adoption, Oral explanation to be held on 11 May 2017 at time 09:00

Documents tabled:
Draft Summary report with response to LoQs

Notes:
There have been 8 designations for this condition: EMEA/OD/005/09 Pegylated recombinant human factor IX, EMEA/OD/117/09 Recombinant fusion protein linking human coagulation factor IX with human albumin, EMA/OD/133/10 Recombinant fusion protein linking human coagulation factor VIIa with human albumin, EMA/OD/090/11 Adeno-associated viral vector containing the human factor IX gene, EMA/OD/041/14 Synthetic double-stranded siRNA oligonucleotide directed against antithrombin mRNA that is covalently linked to a ligand containing three N-acetylgalactosamine residues, EMA/OD/073/14 Recombinant factor VIIa modified with three terminal repeats derived from the β chain of human chorionic gonadotropin, EMA/OD/003/15 Adeno-associated viral vector containing the human factor IX gene, EMA/OD/172/15 Adeno-associated virus viral vector serotype rh10 encoding containing the human factor IX gene

Designations withdrawn: EMEA/OD/008/08 Pegylated recombinant factor VIIa, EMEA/OD/062/09 Sequence modified human recombinant factor VIIa, EMA/OD/070/12 vatreptacog alfa (activated)

2.1.6. - EMA/OD/304/16

Prevention of arterial pulmonary hypertension

Action: For adoption, Oral explanation to be held on 11 May 2017 at 10:00

Documents tabled:
Draft Summary report with response to LoQs

2.1.7. - EMA/OD/001/17

Prevention of rejection following solid organ transplantation

Action: For adoption, Oral explanation to be held on 11 May 2017 at 11:00

Documents tabled:
Draft Summary report with response to LoQs

2.1.8. - EMA/OD/248/16

Prevention of arteriovenous access dysfunction in haemodialysis patients

Action: For adoption, Oral explanation to be held on 11 May 2017 at 12:00

Documents tabled:
Draft Summary report with response to LoQs

Notes:
There has been 1 designation for this condition: EMA/OD/139/13 Recombinant human type I pancreatic elastase

2.1.9. - EMA/OD/016/17

Treatment of distal renal tubular acidosis

Action: For adoption, Oral explanation to be held on 11 May 2017 at 14:30

Documents tabled:
Draft Summary report with response to LoQs

2.1.10. - EMA/OD/014/17

Treatment of ovarian cancer

Action: For adoption, Oral explanation to be held on 11 May 2017 at 15:30

Documents tabled:
Draft Summary report with response to LoQs

Notes:
There have been 31 designations for this condition: EMEA/OD/019/02 Oregovomab, EMEA/OD/061/06 Paclitaxel (micellar), EMEA/OD/080/03 Anti-epithelial cell adhesion molecule/anti-CD3 monoclonal antibody, EMEA/OD/044/03 Trabectedin, 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/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/147/12 Chimeric monoclonal antibody against claudin 6, EMA/OD/039/13 Fosbretabulin tromethamine, EMA/OD/122/13 Trebananib, EMA/OD/186/13 Genetically modified serotype 5/3 adenovirus coding for granulocyte macrophage colony-stimulating factor, EMA/OD/059/14 Cediranib, EMA/OD/281/14 Humanised anti-folate receptor 1 monoclonal antibody conjugated to maytansinoid DM4, EMA/OD/157/14 2-hydroxymethyl-2-methoxymethyl-1-azabicyclo[2,2,2]octan-3-one, EMA/OD/211/14 Chimeric group B adenovirus (11p/3) with deletions in the E3 and E4 regions, EMA/OD/223/14 N-methyl-4-({4-[({3-methyl(methylsulfonyl)amino]pyrazin-2- yl}methyl)amino]-5- (trifluoromethyl)pyrimidin-2-yl}amino)benzamide hydrochloride, EMA/OD/304/14 Human reovirus type 3 Dearing strain, EMA/OD/314/14 {2-amino-8-[4-(pyrrolidinylcarbonyl)phenyl]-(3H-benzo[f]azepin-4-yl)}-N,N-dipropylcarboxamide, 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, EMA/OD/159/16 Vaccine consisting of 5 survivin peptides with different human leukocyte antigen restrictions, EMA/OD/300/16 Poly-cyclodextrin-bis-cysteine-PEG3400-camptothecin-conjugate

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/094/11 Vincaleukoblastin-23-oic acid, O4-deacetyl-2-[(2-mercaptoethoxy)carbonyl]hydrazide, disulfide with N-[4-[[(2-amino-3,4-dihydro-4-oxo-6-pteridinyl)methyl]amino]benzoyl]-L-gamma-glutamyl-L-alpha-aspartyl-L-arginyl-L-alpha-aspartyl-L-alpha-aspartyl-L-cysteine, EMA/OD/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, EMA/OD/114/12 Alisertib

2.1.11. - EMA/OD/311/16

Treatment of neonatal abstinence syndrome

Action: For information

Documents tabled:
Withdrawal request of 26 April 2017

2.1.12. - EMA/OD/020/17

Treatment of subarachnoid haemorrhage (SAH)

Action: For adoption, Oral explanation to be held on 11 May 2017 at 18:00

Documents tabled:
Draft Summary report with response to LoQs

2.1.13. - EMA/OD/007/17

Treatment of tuberous sclerosis

Action: For adoption, Oral explanation to be held on 12 May 2017 at 09:30

Documents tabled:
Draft Summary report with response to LoQs

Notes:
There have been 2 designations for this condition: EMA/OD/010/10 Everolimus, EMA/OD/100/15 Sirolimus

2.1.14. - EMA/OD/008/17

Treatment of sickle cell disease

Action: For adoption

Documents tabled:
Draft Summary report with response to LoQs

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

2.1.15. - EMA/OD/013/17

Treatment of growth hormone deficiency

Action: For adoption

Documents tabled:
Draft Summary report with response to LoQs

Notes:
There have been 2 designations for this condition: EMA/OD/133/12 Recombinant modified human growth hormone, EMA/OD/074/13 Recombinant human growth hormone modified by fusion with two hydrophilic polypeptide chains

2.2. For discussion / preparation for an opinion

2.2.1. - EMA/OD/023/17

Treatment of sudden sensorineural hearing loss

Action: For adoption

Documents tabled:
Draft Summary report

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

2.2.2. - EMA/OD/031/17

Treatment of epidermolysis bullosa

Action: For adoption

Documents tabled:
Draft Summary report

Notes:
There have been 12 designations for this condition: EMEA/OD/111/05 Bilayer engineered skin composed of keratinocytes from the patient (autologous) and fibroblasts from a donor (allogeneic) embedded in a plasma matrix, EMEA/OD/061/09 Allogeneic human dermal fibroblasts, EMA/OD/120/10 Dry extract from birch bark (DER 0.1-0.2:1), extraction solvent n-heptane 95% (V/V), EMA/OD/145/13 Allantoin, EMA/OD/149/13 Diacerein, EMA/OD/201/13 Recombinant human alpha 1 chain homotrimer of type VII collagen, EMA/OD/197/14 Allogeneic adipose-derived adult mesenchymal stem cells contained in a fibrin-based bioengineered dermis, EMA/OD/218/15 Autologous dermal fibroblasts genetically modified ex vivo with a lentiviral vector containing the human COL7A1 gene, EMA/OD/299/14 Ex-vivo-expanded autologous human keratinocytes containing epidermal stem cells transduced with a COL17A1-encoding retroviral vector, EMA/OD/297/14 Ex-vivo-expanded autologous human keratinocytes containing epidermal stem cells transduced with a LAMB3-encoding retroviral vector, EMA/OD/188/15 Ex-vivo-expanded autologous fibroblasts transduced with lentiviral vector containing the COL7A1 gene, EMA/OD/283/16 Ex-vivo-expanded autologous keratinocytes transduced with retroviral vector containing the COL7A1 gene

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

2.2.3. - EMA/OD/028/17

Treatment of C3 glomerulopathy

Action: For adoption

Documents tabled:
Draft Summary report

Notes:
There has been 1 designation for this condition: EMA/OD/104/15 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)

2.2.4. - EMA/OD/295/16

Treatment of invasive candidiasis

Action: For adoption

Documents tabled:
Draft Summary report

2.2.5. - EMA/OD/029/17

Treatment of neuroblastoma

Action: For adoption

Documents tabled:
Draft Summary report

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

2.2.6. - EMA/OD/032/17

Treatment of myotonic disorders

Action: For adoption

Documents tabled:
Draft Summary report

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

2.2.7. - EMA/OD/263/16

Treatment of neonatal abstinence syndrome

Action: For adoption

Documents tabled:
Draft Summary report

2.2.8. - EMA/OD/325/16

Treatment of spinal cord injury

Action: For adoption

Documents 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

2.2.9. - EMA/OD/034/17

Treatment of Creutzfeldt-Jakob disease

Action: For adoption

Documents tabled:
Draft Summary report

2.2.10. - EMA/OD/030/17

Treatment of amyotrophic lateral sclerosis

Action: For adoption

Documents tabled:
Draft Summary report

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

2.2.11. - EMA/OD/024/17

Treatment of mastocytosis

Action: For adoption

Documents tabled:
Draft Summary report

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

2.2.12. - EMA/OD/025/17

Treatment of pachyonychia congenita

Action: For adoption

Documents tabled:
Draft Summary report

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

2.2.13. - EMA/OD/033/17

Treatment of ischemic optic neuropathy

Action: For adoption

Documents tabled:
Draft Summary report

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 tabled:
OMPD applications - appointment of coord. at the 10-12 May 2017 COMP meeting

2.7. Evaluation on-going

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

Action: For information

Notes:
Cross reference to other agenda point. 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 haemophilia A

Action: For adoption

3.1.2. -

Treatment of myasthenia gravis

Action: For adoption

3.1.3. -

Prevention of graft-versus-host disease

Action: For adoption

3.1.4. -

Treatment of mercury toxicity

Action: For adoption

3.1.5. -

Treatment of soft tissue sarcoma

Action: For adoption

3.2. Finalised letters

3.2.1. -

Treatment of Wolfram syndrome

Action: For information

3.2.2. -

Treatment of Wolfram syndrome

Action: For information

3.2.3. -

Treatment of beta-thalassemia intermedia and major

Action: For information

3.3. New requests

3.3.1. -

Treatment of acute hepatic porphyria

Action: For information

3.3.2. -

Treatment of Prader-Willi syndrome

Action: For information

3.3.3. -

Treatment of plasminogen deficiency

Action: For information

3.3.4. -

Treatment of graft-versus-host disease

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. Cuprior - trientine tetrahydrochloride – EMEA/H/C/004005/000, EMA/OD/001/15, EU/3/15/1471

GMP-Orphan SA; Treatment of Wilson's disease

For adoption, Oral explanation to be held on 10 May 2017 at 11:00

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

Notes:
Status of the procedure at the CHMP: Opinion adopted in April 2017

4.1.2. Besponsa - inotuzumab ozogamicin – EMEA/H/C/004119, EMA/OD/194/12, EU/3/13/1127

Pfizer Limited; Treatment of B-cell acute lymphoblastic leukaemia

Action: For adoption, Oral explanation to be held on 10 May 2017 at 15:30

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

Notes:
Status of the procedure at the CHMP: Opinion adopted in April 2017

4.1.3. Spinraza - nusinersen – EMEA/H/C/004312, EMA/OD/141/11, EU/3/12/976

Biogen Idec Ltd; Treatment of 5q spinal muscular atrophy

Action: For information

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

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

4.2.1. - cenegermin - EMEA/H/C/004209, EMA/OD/143/15, EU/3/15/1586

Dompe farmaceutici s.p.a.; Treatment of neurotrophic keratitis

Action: For adoption, Oral explanation to be held on 10 May 2017 at 14:30

Document(s) tabled:

Draft report on review of OMPD

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

Santen Oy; Treatment of vernal keratoconjunctivitis

Action: For discussion

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

4.2.3. Raxone (idebenone) - Type II variation – EMEA/OD/077/06, EU/3/07/437, EMEA/H/C/003834/II/0003

Santhera Pharmaceuticals (Deutschland) GmbH; Treatment of Duchenne muscular dystrophy

CHMP rapporteur: John Joseph Borg; CHMP co-rapporteur: Andrea Laslop

Action: For information

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

4.2.4. – masitinib - EMEA/OD/062/04, EU/3/04/242, EMEA/H/C/004159

AB Science; Treatment of Mastocytosis

Action: For information

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

4.2.5. - vosaroxin – EMA/OD/158/11, EU/3/12/990, EMEA/H/C/004118

Sunesis Europe Ltd; Treatment of acute myeloid leukaemia

Action: For information

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 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 OD for authorised OMP at time 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 10 May 2017 at 13:00

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

7.1.3. Preclinical Models Working Group

Proposed meeting time on 11 May 2017 at 08:30

7.2. Coordination with EMA Scientific Committees or CMDh-v

7.2.1. PDCO/COMP Working Group

Proposed meeting time on 11 May 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 April 2017

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

None

7.4. Cooperation within the EU regulatory network

7.4.1. European Commission

Revision of the Commission Regulation (EC) No 847/2000 of April 2000 laying down the provisions for implementation of the Criteria for designation of a medicinal product as an orphan medicinal product and definitions of the Concept 'similar medicinal product and 'clinical superiority'
Scope: Review of comments received from the public consultation

Action: For discussion

Document(s):
2016_07_EC_consultation_paper

7.5. Cooperation with International Regulators

7.5.1. Food and Drug Administration (FDA)

Action: For information

Document tabled:
Draft Agenda of EMA/FDA teleconference on Orphan Medicines April 18, 2017

7.5.2. Japanese Pharmaceuticals and Medical Devices Agency (PMDA)

None

7.5.3. The Therapeutic Goods Administration (TGA), Australia

None

7.5.4. Health Canada

None

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

None

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/