4 September 2017
EMA/COMP/456162/2017
Inspections, Human Medicines Pharmacovigilance and Committees

Committee for Orphan Medicinal Products (COMP)

Draft agenda for the meeting on 05-07 September 2017

Chair: Bruno Sepodes – Vice-Chair: Lesley Greene

05 September 2017, 09:00-19:00, room 2F

06 September 2017, 08:30-19:00, room 2F

07 September 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 05-07 September 2017. See September 2017 COMP minutes (to be published post October 2017 COMP meeting).

1.2. Adoption of agenda

COMP agenda for 05-07 September 2017.

1.3. Adoption of the minutes

COMP minutes for 11-13 July 2017.

2. Applications for orphan medicinal product designation

2.1. For opinion

2.1.1. - EMA/OD/088/17

Treatment of growth hormone deficiency

Action: For adoption, Oral explanation to be held on 05 September 2017 at 09:30

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

Notes:
There have been 3 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, EMA/OD/013/17 Ibutamoren mesilate

2.1.2. - EMA/OD/089/17

Treatment of pulmonary arterial hypertension

Action: For adoption, Oral explanation to be held on 05 September 2017 at 14:30

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

Notes:
There have been 5 designations for this condition: EMEA/OD/018/08 Beraprost sodium, EMA/OD/023/11 Macitentan, EMA/OD/111/11 Sodium nitrite, EMA/OD/179/15 Ubenimex, EMA/OD/299/16 (S)-8-{2-amino-6-[1-(5-chloro-biphenyl-2-yl)-(R)-2,2,2-trifluoro-ethoxy]-pyrimidin-4-yl}-2,8-diaza-spiro[4.5]decane-3-carboxylic acid ethyl ester)

2.1.3. - EMA/OD/292/16

Treatment of ATTR amyloidosis

Action: For information

Document(s) tabled:
Withdrawal request of 15 August 2017

Notes:
There has been 1 designation for this condition: EMA/OD/098/13 Phosphorothioate oligonucleotide targeted to transthyretin

Designation withdrawn: EMA/OD/194/13 Synthetic double-stranded siRNA oligonucleotide directed against transthyretin mRNA and covalently linked to a ligand containing three N-acetylgalactosamine residues

2.1.4. - EMA/OD/090/17

Treatment of glioblastoma

Action: For information

Document(s) tabled:
Withdrawal request of 15 August 2017

Notes:
Designation withdrawn: EMEA/OD/012/00 Fluorouracil

2.1.5. - EMA/OD/059/17

Treatment of short bowel syndrome

Action: For adoption

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

Notes:
There have been 3 designations for this condition: EMEA/OD/045/01 [gly2]-recombinant human glucagon-like peptide, EMA/OD/080/14 Oxalobacter formigenes strain HC-1, EMA/OD/050/15 Insulin human (rDNA)

2.1.6. - EMA/OD/039/17

Treatment of partial deep dermal and full thickness burns

Action: For adoption, clarification TC on the written responses to be held on 05 September 2017 at 18:00

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

Notes:

There have been 3 designations for this condition: EMEA/OD/012/02 Purified bromelain, EMA/OD/163/15 Bilayer, engineered, collagen hydrogel-based skin graft composed of autologous keratinocytes and fibroblasts, EMA/OD/068/16 Sodium hypochlorite

2.1.7. - EMA/OD/091/17

Treatment of growth hormone deficiency

Action: For adoption, Oral explanation to be held on 06 September 2017 at 09:30

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

Notes:
There have been 3 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, EMA/OD/013/17 Ibutamoren mesilate

2.1.8. - EMA/OD/079/17

Treatment of primary sclerosing cholangitis

Action: For adoption, Oral explanation to be held on 06 September 2017 at 11:00

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

Notes:
There have been 5 designations for this condition: EMA/OD/127/13 (4R,5R)-1-[[4-[[4-[3,3-dibutyl-7-(dimethylamino)-2,3,4,5-tetrahydro-4-hydroxy-1,1-dioxido-1-benzothiepin-5-yl]phenoxy]methyl]phenyl]methyl]-4-aza-1-azoniabicyclo[2.2.2]octane chloride, EMA/OD/136/13 Obeticholic acid, EMA/OD/026/14 Norursodeoxycholic acid, EMA/OD/288/14 Recombinant human monoclonal antibody binding to vascular adhesion protein-1, EMA/OD/139/15 Variant of recombinant human fibroblast growth factor 19

2.1.9. - EMA/OD/076/17

Treatment of infantile spasms

Action: For adoption, Oral explanation to be held on 06 September 2017 at 12:00

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

2.1.10. - EMA/OD/097/17

Treatment of neurodegeneration with brain iron accumulation

Action: For adoption, Oral explanation to be held on 06 September 2017 at 14:30

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

2.1.11. - EMA/OD/060/17

Treatment of Leber congenital amaurosis

Action: For adoption, Oral explanation to be held on 06 September 2017 at 15:30

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

Notes:
There have been 3 designations for this condition: EMA/OD/182/13 Adeno-associated viral vector serotype 8 containing the human GUCY2D gene, EMA/OD/163/10 9-cis-Retinyl acetate, EMA/OD/150/11 Adenovirus associated viral vector serotype 2 containing the human RPE65 gene

Designations withdrawn: EMA/OD/063/11 Adeno-associated viral vector serotype 8 containing the human AIPL1 gene

2.1.12. - EMA/OD/035/17

Treatment of ovarian cancer

Action: For adoption, Oral explanation to be held on 06 September 2017 at 17:00

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

Notes:
There have been 30 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/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 …, 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, EMA/OD/314/14 {2-amino-8-[4-(pyrrolidinylcarbonyl)phenyl]-(3H-benzo[f]azepin-4-yl)}-N,N-dipropylcarboxamide

2.1.13. - EMA/OD/087/17

Treatment of mastocytosis

Action: For adoption

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

2.2. For discussion / preparation for an opinion

2.2.1. - EMA/OD/102/17

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/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, EMA/OD/008/17 Decitabine and tetrahydrouridine

Designations withdrawn: EMA/OD/162/12 Poloxamer 188, EMA/OD/249/14 5-hydroxymethyl-2-furfural

2.2.2. - EMA/OD/120/17

Treatment of subarachnoid haemorrhage (SAH)

Action: For discussion/adoption

Document(s) tabled:
Draft Summary report

2.2.3. - EMA/OD/119/17

Treatment of gastrointestinal stromal tumours

Action: For discussion/adoption

Document(s) tabled:
Draft Summary report

Notes:
There have been 2 designations for this condition: EMA/OD/037/17 (S)-1-(4-fluorophenyl)-1-(2-(4-(6-(1-methyl-1H-pyrazol-4-yl)pyrrolo[2,1-f][1,2,4]triazin-4-yl)piperazin-yl)pyrimidin-5-yl)ethan-1-amine, EMA/OD/328/16 177Lu-DOTA-pABzA-DIG-dPhe-Gln-Trp-Ala-Val-Gly-His-NHCH[(CH2-CH(CH3)2]2

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

2.2.4. - EMA/OD/124/17

Treatment of biliary tract cancer

Action: For discussion/adoption

Document(s) tabled:
Draft Summary report

Notes:
There have been 2 designations for this condition: EMA/OD/305/14 5,10,15,20-tetrakis(2,6-difluoro-3-N-methylsulfamoylphenyl)bacteriochlorin, EMA/OD/245/15 (R)-6-(2-fluorophenyl)-N-(3-(2-((2-methoxyethyl)amino)ethyl)phenyl)-5,6-dihydrobenzo[h]quinazolin-2-amine dihydrochloride

2.2.5. - EMA/OD/111/17

Treatment of pancreatic cancer

Action: For discussion/adoption

Document(s) tabled:
Draft Summary report

Notes:
There have been 36 designations for this condition: EMEA/OD/055/02 G17(9) gastrin-Diphtheria Toxoid Conjugate, EMEA/OD/068/02 Rubitecan, EMEA/OD/009/05 Bovine bile extract, EMEA/OD/028/05 4-imino-1, 3-diazobicyclo-[3.1.0]-hexan-2-one, EMEA/OD/063/06 Paclitaxel (liposomal), EMEA/OD/026/06 Human telomerase reverse transcriptase peptide (611-626), EMEA/OD/103/06 Cisplatin (liposomal), EMEA/OD/100/08 L-asparaginase encapsulated in erythrocytes, EMEA/OD/006/08 Nimotuzumab, EMEA/OD/080/08 Yttrium (90Y)-DOTA-radiolabelled humanized monoclonal antibody against mucin 1, EMEA/OD/101/08 S-[2,3-bispalmitoyloxy-(2R)-propyl]-cysteinyl-GNNDESNISFKEK, EMEA/OD/030/09 Trabedersen, EMEA/OD/105/09 Brivudine, EMEA/OD/069/09 N-[(2S)-2,3-dihydroxypropyl]-3-[(2-fluoro-4-iodophenyl)amino]isonicotinamide hydrochloride, EMEA/OD/063/09 Masitinib mesilate, EMA/OD/135/10 Glufosfamide, EMA/OD/150/10 Salirasib, EMA/OD/007/11 Mixture of seven synthetic fragments consisting of p21 RAS peptides, EMA/OD/008/11 Genetically modified human adenovirus encoding human PH20 hyaluronidase, EMA/OD/051/11 Nanoliposomal irinotecan, EMA/OD/065/12 Mixture of two allogeneic human pancreatic cancer cell lines stably transduced with a retroviral vector encoding the murine alpha-(1,3)-galactosyltransferase gene, EMA/OD/037/13 (1-methyl-2-nitro-1H-imidazole-5-yl)methyl N,N'-bis(2-bromoethyl)diamidophosphate, EMA/OD/071/13 Chimeric monoclonal antibody against claudin-18 splice variant 2, EMA/OD/164/13 Cysteamine bitartrate, EMA/OD/081/14 Immunoglobulin G1, anti-(human tumour-associated calcium signal transducer 2)(human-Mus musculus monoclonal hRS7 heavy chain), disulfide with human-Mus musculus monoclonal hRS7-chain, dimer, hexakis(thioether) with (4S)-4-[[[[4-[[(2S)-2-(4-aminobutyl)-2-[[2-[2-[[26-[4-[[[[4-[(3-mercapto-2,5-dioxo-1-pyrrolidinyl)methyl]cyclohexyl]carbonyl]amino]methyl]-1H-1,2,3-triazol-1-yl]-3,6,9,12,15,18,21,24-octaoxahexacos-1-yl]amino]-2-oxoethoxy]acetyl]amino]-1-oxoethyl]amino]phenyl]methoxy]carbonyl]oxy]-4,11-diethyl-9-hydroxy-1H-pyrano[3',4':6,7]indolizino[1,2-b]quinoline-3,14(4H,12H)-dione, EMA/OD/085/14 [5-Amino-1-(4-fluoro-phenyl)-1H-pyrazol-4-yl]-[3-(2,3-dihydroxy-propoxy)-phenyl]-methanone, EMA/OD/187/14 Herpes simplex type 1 virus containing cellular B-myb gene as tumour-specific promoter, EMA/OD/143/14 Heat-killed Mycobacterium obuense (whole cell), EMA/OD/173/14 Pegylated recombinant human hyaluronidase PH20, EMA/OD/302/14 Human reovirus type 3 Dearing strain, EMA/OD/034/15 Modified adenovirus serotype 5/35 containing a CMV promoter-driven transgene cassette with the human transgenes for a membrane-bound CD40 ligand (TMZ-CD40L) and full length 4-1BBL, EMA/OD/168/15 Live attenuated Listeria monocytogenes delta actA/delta inlB strain expressing human mesothelin, EMA/OD/169/15 Two allogenic irradiated pancreatic tumour cell lines, EMA/OD/193/16 Pegylated recombinant human interleukin-10, EMA/OD/241/16 Antroquinonol, EMA/OD/273/16 Humanised IgG4 monoclonal antibody to the human toll-like receptor type 2

Designations withdrawn: EMEA/OD/070/02 Iodine (131I) Anti-CEA sheep-human chimeric monoclonal antibody, EMEA/OD/040/04 Deuterium oxide, EMEA/OD/097/05 26 base single stranded phosphodiester DNA oligonucleotide, EMEA/OD/111/07 Chimeric antibody to mesothelin, EMEA/OD/067/09 5'-O-(trans-9''-octadecenoyl)-1-beta-D-2'deoxy-2',2'-difluorocytidine, EMA/OD/087/10 Nanoparticle albumin-bound paclitaxel, EMA/OD/007/12 Polyinosine-polycytidylic acid coupled with the polycationic polyethyleneimine,

EMA/OD/145/12 Recombinant human monoclonal antibody of the IgG1 kappa class against prostate stem cell antigen

2.2.6. - EMA/OD/125/17

Treatment of plasma cell myeloma

Action: For discussion/adoption

Document(s) tabled:
Draft Summary report

Notes:
There have been 9 designations for this condition: EMA/OD/072/13 Recombinant human monoclonal IgM antibody targeting glucose-regulated protein 78, EMA/OD/038/13 Daratumumab, EMA/OD/198/13 humanized monoclonal antibody against CD38, EMA/OD/035/14 Marizomib, EMA/OD/087/14 Selinexor, EMA/OD/293/14 Melphalan flufenamide, EMA/OD/214/14 Synthetic signal peptide of human Mucin-1 (amino acids 1-21), EMA/OD/277/14 Reduced oxidised N-acetyl heparin, EMA/OD/274/16 Autologous T-cells transduced with lentiviral vector encoding an anti-SLAMF7 CD28/CD3-zeta chimeric antigen receptor

2.2.7. - EMA/OD/112/17

Treatment of beta-thalassaemia intermedia and major

Action: For discussion/adoption

Document(s) tabled:
Draft Summary report

Notes:
There have been 6 designations for this condition: EMEA/OD/098/08 2,2-dimethylbutyric acid, sodium salt, EMEA/OD/111/08 Autologous haematopoietic stem cells transduced with lentiviral vector encoding the human beta-globin gene, EMA/OD/047/14 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/142/15 Sirolimus, EMA/OD/146/12 Autologous CD34+ haematopoietic stem cells transduced with lentiviral vector encoding the human βA-T87Q-globin gene, EMA/OD/189/14 Benserazide hydrochloride

2.2.8. - EMA/OD/105/17

Treatment in solid organ transplantation

Action: For discussion/adoption

Document(s) tabled:
Draft Summary report

Notes:
There have been 2 designations for this condition: EMA/OD/286/16 Rituximab, EMA/OD/287/16 Human normal immunoglobulin

2.2.9. - EMA/OD/100/17

Treatment of Rett syndrome

Action: For discussion/adoption

Document(s) tabled:
Draft Summary report

Notes:
There have been 4 designations for this condition: EMEA/OD/001/09 Desipramine chlorhydrate, EMA/OD/163/13 3-Chloro-4-fluorophenyl-[4-fluoro-4-{[(5-methylpyrimidin-2-ylmethyl) amino]methyl}piperidin-1-yl]methanone, EMA/OD/056/15 Glycyl-L-2-methylprolyl-L-glutamic acid, EMA/OD/058/15 Sarizotan hydrochloride

2.2.10. - EMA/OD/116/17

Treatment of haemophilia B

Action: For discussion/adoption

Document(s) tabled:
Draft Summary report

Notes:
There have been 8 designations for this condition: 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, EMA/OD/018/17 Recombinant human factor IX protein modified with three point mutations

Designations withdrawn: EMEA/OD/008/08 Pegylated recombinant factor VIIa, EMEA/OD/005/09 Pegylated recombinant human factor IX, EMEA/OD/062/09 Sequence modified human recombinant factor VIIa, EMA/OD/070/12 vatreptacog alfa (activated)

2.2.11. - EMA/OD/115/17

Treatment of Prader-Willi-Syndrome (PWS)

Action: For discussion/adoption

Document(s) tabled:
Draft Summary report

Notes:
There have been 3 designations for this condition: EMA/OD/054/14 Oxytocin, EMA/OD/033/16 Setmelanotide, EMA/OD/119/11 Carbetocin

Designations withdrawn: EMA/OD/023/14 Beloranib

2.2.12. - EMA/OD/117/17

Treatment of beta-thalassaemia intermedia and major

Action: For discussion/adoption

Document(s) tabled:
Draft Summary report

Notes:
There have been 6 designations for this condition: Please see 2.2.7.

2.2.13. - EMA/OD/040/17

Treatment of acute myeloid leukaemia

Action: For discussion/adoption

Document(s) tabled:
Draft Summary report

Notes:
There have been 50 designations for this condition: EMEA/OD/022/00 Gemtuzumab ozogamicin, EMEA/OD/028/04 Midostaurin, EMEA/OD/056/06 Antisense oligonucleotide 5'-d[P-Thio] (CCCTG CTCCC CCCTG GCTCC)-3' (see comments box for cenersen sodium), EMEA/OD/098/04 Tipifarnib, EMEA/OD/094/04 Histamine dihydrochloride, EMEA/OD/066/05 1,2-bis(methylsulphonyl)-1-(2-chloroethyl)-2-[(methylamino)carbonyl]hydrazine, EMEA/OD/100/05 zosuquidar trihydrochloride, EMEA/OD/004/06 Decitabine, EMEA/OD/049/07 5'-O-(trans-9"-octadecenoyl)-1-ß-D-arabinofuranosyl cytosine, EMEA/OD/087/07 Recombinant human histone H1.3 and recombinant human N-bis-met-histone H1.3, EMEA/OD/085/07 Azacitidine, EMEA/OD/099/07 N- (2-Amino-phenyl)-4-[(4-pyridin-3-yl-pyrimidin-2-ylamino)-methyl] benzamide, EMEA/OD/118/07 Ribonucleotide reductase R2 specific phosphorothioate oligonucleotide, EMEA/OD/015/08 Sapacitabine, EMEA/OD/048/08 Daunorubicin (liposomal), EMEA/OD/105/08 N-(5-tert-Butylisoxazol-3-yl)-N'-{4-[7-(2-(morpholin-4-yl)ethoxy) imidazo[2,1-b][1,3]benzothiazol-2-yl]phenyl}urea di-hydrochloride salt, EMEA/OD/028/09 Tosedostat, EMEA/OD/091/09 1-Cyclopropyl-3-[3-(5-morpholin-4-ylmethyl-1H-benzoimidazol-2-yl)-1H-pyrazol-4-yl]-urea, EMEA/OD/147/09 2-methoxymethyl-2-hydroxymethyl-1-azabicyclo[2,2,2]octan-3-one, EMA/OD/044/10 Allogeneic T cells encoding an exogenous TK gene, EMA/OD/094/10 N-[(2S)-2,3-dihydroxypropyl]-3-[(2-fluoro-4-iodophenyl) amino] isonicotinamide hydrochloride, EMA/OD/101/11 Allogeneic human dendritic cells derived from a CD34+ progenitor cell line, EMA/OD/070/11 Liposomal combination of cytarabine and daunorubicin, EMA/OD/158/11 Vosaroxin, EMA/OD/167/12 L-asparaginase encapsulated in erythrocytes, EMA/OD/064/13 trans-N1-((1R,2S)-2-phenylcyclopropyl)cyclohexane-1,4-diamine bis-hydrochloride, EMA/OD/141/13 (2R,3R,4S,5R)-2-(6-amino-9H-purin-9-yl)-5-((((1r,3S)-3-(2-(5-(tert-butyl)-1Hbenzo[d]imidazol-2-yl)ethyl)cyclobutyl)(isopropyl) amino)methyl)tetrahydrofuran-3,4-diol, EMA/OD/181/13 Volasertib, EMA/OD/100/14 4-{[(2R,3S,4R,5S)-4-(4-Chloro-2-fluoro-phenyl)-3-(3-chloro-2-fluoro-phenyl)-4-cyano-5-(2,2-dimethyl-propyl)-pyrrolidine-2-carbonyl]-amino}-3-methoxy-benzoic acid, EMA/OD/061/14 (Z)-3-(3-(3,5-bis(trifluoromethyl)phenyl)-1H-1,2,4-triazol-1-yl)-N'-(pyrazin-2-yl)acrylohydrazide,

EMA/OD/103/14 Donor T lymphocytes depleted ex vivo of host alloreactive T cells using photodynamic treatment, EMA/OD/175/14 Allogeneic ex vivo-generated natural killer cells from CD34+ umbilical cord blood progenitor cells, EMA/OD/240/14 Alvocidib, EMA/OD/188/14 Allogeneic, umbilical cord blood-derived, ex vivo-expanded, haematopoietic CD133+ cells / allogeneic, umbilical cord blood-derived, non-expanded, haematopoietic CD133- cells, EMA/OD/258/14 Ulocuplumab, EMA/OD/045/15 inecalcitol, EMA/OD/037/15 2-((3-((4-((3-aminopropyl)amino)butyl)amino)propyl)amino)-N-((5S,5aS,8aR,9R)-9-(4-hydroxy-3,5-dimethoxyphenyl)-8-oxo-5,5a,6,8,8a,9-hexahydrofuro[3',4':6,7]naphtho[2,3-d][1,3]dioxol-5-yl)acetamide, tetrahydrochloride, EMA/OD/089/15 CD33-directed antibody-drug conjugate consisting of an antibody conjugated to a DNA cross-linking pyrrolobenzodiazepine dimer drug, EMA/OD/112/15 Recombinant human interleukin-3 truncated diphtheria toxin fusion protein, EMA/OD/145/15 Humanised monoclonal antibody of the IgG4 kappa isotype targeting CD47, EMA/OD/165/15 Sodium (2R,3S,5R)-5-(4-amino-2-oxo-1,3,5-triazin-1(2H)-yl)-2-(hydroxymethyl)tetrahydrofuran-3-yl ((2R,3S,5R)-5-(2-amino-6-oxo-1H-purin-9(6H)-yl)-3-hydroxytetrahydrofuran-2-yl)methyl phosphate, EMA/OD/144/15 Combretastatin A1-diphosphate, EMA/OD/180/15 Arsenic trioxide, EMA/OD/205/15 Venetoclax, EMA/OD/233/15 Tyr-Met-Phe-Pro-Asn-Ala-Pro-Tyr-Leu, Ser-Gly-Gln-Ala-Tyr-Met-Phe-Pro-Asn-Ala-Pro-Tyr-Leu-Pro-Ser-Cys-Leu-Glu-Ser, Arg-Ser-Asp-Glu-Leu-Val-Arg-His-His-Asn-Met-His-Gln-Arg-Asn-Met-Thr-Lys-Leu and Pro-Gly-Cys-Asn-Lys-Arg-Tyr-Phe-Lys-Leu-Ser-His-Leu-Gln-Met-His-Ser-Arg-Lys-His-Thr-Gly, EMA/OD/253/15 2-methyl-1-[(4-[6-(trifluoromethyl)pyridin-2-yl]-6-{[2-(trifluoromethyl)pyridin-4-yl]amino}-1,3,5-triazin-2-yl)amino]propan-2-ol methanesulfonate, EMA/OD/155/16 P-ethoxy growth factor receptor-bound protein 2 (Grb2) antisense oligonucleotide, EMA/OD/197/16 Ivosidenib, EMA/OD/319/16 225Ac-lintuzumab, EMA/OD/010/17 Sodium (1R, 3R, 4R, 5S)-3-({2-N-acetylamino-2-deoxy-3-O-[(1S)-1-carboxylato-2-cyclohexylethyl]-β-D-galactopyranosyl}oxy)-4-({6-deoxy-α-L-galactopyranosyl}oxy)-5-ethyl-cyclohexan-1-yl-(38-oxo-2,5,8,11,14,17,20,23,26,29,32,35-dodecaoxa-39-azahentetracontan-41-yl) carboxamide

Designations withdrawn: EMEA/OD/065/02 2-chloro-9-[2-deoxy-2-fluoro-ß-D-arabinofuranosyl]adenine, EMEA/OD/051/04 Homoharringtonine, EMEA/OD/059/04 Val-Leu-Gln-Glu-Leu-Asn-Val-Thr-Val (Pr1 nanopeptide, sequence 169-177, of proteinase 3), EMEA/OD/045/05 Troxacitabine, EMEA/OD/018/06 Human monoclonal antibody against inhibitory killer cell lg-like receptors (1-7 F9), EMEA/OD/020/06 Lestaurtinib, EMEA/OD/024/07 Arsenic trioxide, EMEA/OD/069/07 Amonafide L-malate, EMEA/OD/060/08 2-[[3-({4-[(5-{2-[(3-Fluorophenyl)amino]-2-oxoethyl}-1H-pyrazol-3-yl)amino]-quinazolin-7-yl}oxy)propyl](ethyl)amino]ethyl dihydrogen phosphate trihydrate, EMEA/OD/118/08 Lintuzumab, EMEA/OD/090/08 Allogeneic ex vivo expanded umbilical cord blood cells, EMEA/OD/016/09 26 base single stranded phosphodiester DNA oligonucleotide, EMEA/OD/132/09 (1S, 2S, 3R, 4R)-3-(5-Fluoro-2-(3-methyl-4-(4-methylpiperazin-1-yl)-phenylamino)-pyrimidin-4-ylamino)-bicyclo[2.2.1]hept-5-ene-2-carboxamide benzoate), EMA/OD/023/10 1-[2-(Benzo[1,2,5]thiadiazol-5-ylamino)-6-(2,6-dichloro-phenyl)-pyrido[2,3-d]pyrimidin-7-yl]-3-tert-butyl-urea, EMA/OD/161/10 Allogeneic bone marrow stem cells treated ex vivo with 16,16-dimethyl prostaglandin E2, EMA/OD/156/10 Allogeneic umbilical cord blood cells treated ex vivo with 16,16-dimethyl prostaglandin E2, EMA/OD/067/11 1-(4-{4-amino-7-[1-(2-hydroxyethyl)-1H- pyrazol-4-yl]thieno[3,2-c]pyridin-3-yl}phenyl)-3-(3-fluorophenyl)urea, EMA/OD/105/12 Liposomal daunorubicin

2.2.14. - EMA/OD/106/17

Treatment of acute myeloid leukaemia

Action: For discussion/adoption

Document(s) tabled:
Draft Summary report

Notes:
There have been 50 designations for this condition: Please see 2.2.13.

2.2.15. - EMA/OD/103/17

Treatment of follicular lymphoma

Action: For discussion/adoption

Document(s) tabled:
Draft Summary report

Notes:
There have been 8 designations for this condition: EMEA/OD/040/06 Autologous tumor-derived immunoglobulin idiotype coupled to keyhole limpet haemocyanin, EMEA/OD/065/04 Recombinant hisitidine-tagged idiotype immunoglobulin Fab fragment of clonal B-cell receptors, EMA/OD/158/12 lenalidomide, EMA/OD/047/13 (S)-3-(1-(9H-purin-6-ylamino)ethyl)-8-chloro-2-phenylisoquinolin-1(2H)-one, EMA/OD/111/13 Ibrutinib, EMA/OD/200/13 177Lu-tetraxetan-tetulomab, EMA/OD/013/15 obinutuzumab, EMA/OD/135/15 Autologous T cells transduced with retroviral vector encoding an anti-CD19 CD28/CD3-zeta chimeric antigen receptor

Designations withdrawn: EMEA/OD/061/02 Iodine (131I) tositumomab, EMEA/OD/079/02 Tositumomab, EMA/OD/053/13 Idelalisib

2.2.16. - EMA/OD/077/17

Treatment of multiple myeloma

Action: For discussion/adoption

Document(s) tabled:
Draft Summary report

Notes:
There have been 15 designations for this condition: EMEA/OD/040/01 Thalidomide, EMEA/OD/063/03 3-(4'aminoisoindoline-1'-one)-1-piperidine-2,6-dione, EMEA/OD/044/04 Aplidine, EMEA/OD/066/04 Recombinant histidine-tagged idiotype immunoglobulin Fab fragment of clonal B-cell receptors, EMEA/OD/012/05 N-(methyl-diazacyclohexyl-methylbenzamide)-azaphenyl-aminothiopyrrole, EMEA/OD/120/07 Carfilzomib, EMEA/OD/068/08 N2'-Deacetyl-N2'-[4-methyl-4-(oxobuthyldithio)-1-oxopentyl]-maytansine-chimerized anti-CD138 IgG4 monoclonal antibody, EMEA/OD/076/08 Human anti-intercellular adhesion molecule-1 monoclonal antibody, EMEA/OD/053/08 Milatuzumab, EMEA/OD/053/09 Pomalidomide, EMA/OD/017/11 Acadesine, EMA/OD/048/11 2,2'-{2-[(1R)-1-({[(2,5-dichlorobenzoyl)amino]acetyl}amino)-3-methylbutyl]-5-oxo-1,3,2-

dioxaborolane-4,4-diyl}diacetic acid, EMA/OD/113/12 Panobinostat, EMA/OD/121/16 Venetoclax, EMA/OD/270/16 Autologous T lymphocyte-enriched population of cells transduced with a lentiviral vector encoding a chimeric antigen receptor targeting human B cell maturation antigen with 4-1BB and CD3-zeta intracellular signalling domains

Designations withdrawn: EMEA/OD/048/00 Arsenic trioxide, EMEA/OD/003/01 Humanised anti-HM1.24 monoclonal antibody, EMEA/OD/018/00 Thalidomide, EMEA/OD/026/01 Deoxyribose phosphorothioate (5'-tct-ccc-agc-gtg-cgc-cat-3'), EMEA/OD/019/01 Thalidomide, EMEA/OD/070/04 17-allylamino-17-demethoxygeldanamycin, EMEA/OD/093/05 Human monoclonal antibody against HLA-DR, EMEA/OD/003/09 Chimeric-anti-interleukin-6 monoclonal antibody, EMEA/OD/133/09 Dexamethasone (40 mg tablet), EMEA/OD/130/09 Perifosine, EMA/OD/115/10 Maytansinoid-conjugated humanised monoclonal antibody against CD56, EMA/OD/137/10 Vorinostat, EMA/OD/137/11 Chimeric monoclonal antibody against kappa myeloma antigen, EMA/OD/061/12 Elotuzumab

2.2.17. - EMA/OD/109/17

Treatment of chronic thromboembolic pulmonary hypertension (CTEPH)

Action: For discussion/adoption

Document(s) tabled:
Draft Summary report

Notes:
There has been 1 designation for this condition: EMA/OD/154/12 Treprostinil sodium

2.2.18. - EMA/OD/113/17

Treatment of haemophilia B

Action: For discussion/adoption

Document(s) tabled:
Draft Summary report

Notes:
There have been 8 designations for this condition: Please see 2.2.10.

2.2.19. - EMA/OD/127/17

Treatment of subarachnoid hemorrhage

Action: For discussion/adoption

Document(s) tabled:
Draft Summary report

2.2.20. - EMA/OD/121/17

Treatment of peripheral T-cell lymphoma

Action: For discussion/adoption

Document(s) tabled:

Draft Summary report

Notes:
There has been 1 designation for this condition: EMA/OD/131/16 Fenretinide

2.2.21. - EMA/OD/055/17

Treatment of Charcot-Marie-Tooth disease

Action: For discussion/adoption

Document(s) tabled:
Draft Summary report

Notes:
There has been 1 designation for this condition: EMA/OD/170/15 2-(2-chlorobenzylidene)hydrazinecarboximidamide acetate

2.2.22. - EMA/OD/101/17

Treatment of acute myeloid leukaemia

Action: For discussion/adoption

Document(s) tabled:
Draft Summary report

Notes:
There have been 50 designations for this condition: Please see 2.2.13.

2.2.23. - EMA/OD/126/17

Treatment of microvillus inclusion disease

Action: For discussion/adoption

Document(s) tabled:
Draft Summary report

2.2.24. - EMA/OD/114/17

Treatment of primary biliary cholangitis

Action: For discussion/adoption

Document(s) tabled:
Draft Summary report

2.2.25. - EMA/OD/104/17

Treatment in solid organ transplantation

Action: For discussion/adoption

Document(s) tabled:
Draft Summary report

Notes:
There have been 2 designations for this condition: Please see 2.2.8.

2.2.26. - EMA/OD/066/17

Treatment of Prader-Willi syndrome

Action: For discussion/adoption

Document(s) tabled:
Draft Summary report

Notes:
There have been 3 designations for this condition: Please see 2.2.11.

2.2.27. - EMA/OD/067/17

Treatment of pancreatic cancer

Action: For discussion/adoption

Document(s) tabled:
Draft Summary report

Notes:
There have been 36 designations for this condition: Please see 2.2.5.

2.2.28. - EMA/OD/107/17

Treatment of idiopathic pulmonary fibrosis

Action: For discussion/adoption

Document(s) tabled:
Draft Summary report

Notes:
There have been 12 designations for this condition: 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/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, EMA/OD/046/16 3-[4-(1H-imidazol-1-ylmethyl)phenyl]-5-(2-methylpropyl)thiophene-2-[(N-butyloxylcarbamate)-sulphonamide] sodium salt, EMA/OD/088/16 2-((2-ethyl-6-(4-(2-(3-hydroxyazetidin-1-yl)-2-oxoethyl)-piperazin-1-yl)-8-methylimidazo[1,2-alpha]pyridin-3-yl)-(methyl)amino)-4-(4-fluorophenyl)-thiazole-5-carbonitrile

Designations withdrawn: EMEA/OD/002/05 Interferon gamma, EMEA/OD/033/04 Heparin-Sodium, 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, EMA/OD/111/12 Tralokinumab

2.2.29. - EMA/OD/110/17

Treatment of focal segmental glomerulosclerosis

Action: For discussion/adoption

Document(s) tabled:
Draft Summary report

Notes:
There has been 1 designation for this condition: EMA/OD/115/15 4'-[(2-Butyl-4-oxo-1,3-diazaspiro[4.4]non-1-en-3-yl)methyl]-N-(4,5-dimethyl-3-isoxazolyl)-2'-(ethoxymethyl)-[1,1'-biphenyl]-2-sulfonamide

Designation withdrawn: EMA/OD/146/10 Fresolimumab

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 05-07 September 2017 COMP meeting

2.7. Evaluation on-going

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

Action: For information

Notes:
See 7.8.1. Evaluation Ongoing.

3. Requests for protocol assistance with significant benefit question

3.1. Ongoing procedures

3.1.1. -

Treatment of paroxysmal nocturnal haemoglobinuria

Action: For adoption

3.1.2. -

Treatment of congenital factor VII deficiency

Action: For adoption

3.1.3. -

Treatment of myelodysplastic syndromes

Action: For adoption

3.1.4. -

Treatment in solid organ transplantation

Action: For adoption

3.2. Finalised letters

3.2.1. -

Treatment of acute hepatic porphyria

Action: For information

3.2.2. -

Treatment of Prader-Willi syndrome

Action: For information

3.2.3. -

Treatment of haemophilia A

Action: For information

3.3. New requests

None

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

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

Alexion Europe SAS; Treatment of myasthenia gravis

CHMP rapporteur: Jorge Camarero Jiménez; CHMP co-rapporteur: Alexandre Moreau

Action: For information

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

4.2.1. Masipro – Masitinib – EMEA/OD/062/04, EU/3/04/242, EMEA/H/C/004159

AB Science; Treatment of Mastocytosis

Action: For information

4.2.2. - Letermovir –EMA/OD/090/10, EU/3/11/849, EMEA/H/C/004536

Merck Sharp & Dohme Limited; Prevention of cytomegalovirus disease in patients with impaired cell-mediated immunity deemed at risk

Action: For discussion

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

4.2.3. - Niraparib – EMA/OD/015/10, EU/3/10/760,EMEA/H/C/004249

Tesaro UK Limited; Treatment of ovarian cancer

Action: For discussion

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

4.2.4. 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

4.3. 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/0018

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

CHMP rapporteur: Alexandre Moreau

Action: For information

Documents tabled:
Draft report on review of OMPD

5.2.2. Nplate - Recombinant megakaryopoiesis-stimulating protein Romiplostim –Type II variation - EMEA/OD/008/05, EU/3/05/283, EMEA/H/C/000942/II/0060/G

Amgen Europe BV; Treatment of idiopathic thrombocytopenic purpura

CHMP rapporteur: Concepcion Prieto Yerro; CHMP co-rapporteur: Paula Boudewina van Hennik

Action: For information

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

5.2.3. Bosulif – Bosutinib –Type II variation - EMEA/OD/160/09, EU/3/10/762, EMEA/H/C/002373/II/0025/G

Pfizer Limited; Treatment of chronic myeloid leukaemia

CHMP rapporteur: Harald Enzman

Action: For information

5.2.4. Kalydeco – Ivacaftor–Type II variation - EMEA/OD/010/08, EU/3/08/556, EMEA/H/C/002494/II/0063/G

Vertex Pharmaceuticals; Treatment of cystic fibrosis

CHMP rapporteur: Concepcion Prieto Yerro

Action: For information

5.2.5. Inovelon – Rufinamide–Type II variation - EMEA/OD/047/04, EU/3/04/240, EMEA/H/C/000660/II/0045

Eisai Ltd; Treatment of Lennox-Gastaut syndrome

CHMP rapporteur: Alexandre Moreau

Action: For information

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. COMP Strategic Review & Learning meeting, 19-21 September 2017, Lisbon, Portugal

Action: For information

Document(s) tabled:
Final agenda

7.1.2. Protocol Assistance Working Group

Proposed meeting time on 05 September 2017 at 13:00

Document(s) tabled:
PAWG draft agenda for September 2017 meeting
PAWG draft minutes for July 2017 meeting

7.1.3. Non-Clinical Working Group

Proposed meeting time on 06 September 2017 at 08:30

7.1.4. Condition Working Group

Proposed meeting time on 07 September 2017 at 08:30

7.2. Coordination with EMA Scientific Committees or CMDh-v

7.2.1. Recommendations on eligibility to PRIME – report from CHMP

Action: For information

Document(s) tabled:
PRIME eligibility requests - list of adopted outcomes July 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 – 19/20 September 2017

Action: For information

Document(s) tabled:
Personalised medicines workshop report (EMA/185440/2017)
Draft Agenda AMR workshop – 19 September 2017 (EMA/765134/2016)
Draft agenda of the PCWP/HCPWP meeting - 20 September 2017 (EMA/370525/2017)

7.4. Cooperation within the EU regulatory network

7.4.1. European Commission

None

7.5. Cooperation with International Regulators

7.5.1. Food and Drug Administration (FDA)

Action: For information

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. EMA Business Pipeline activity and Horizon scanning

Action: For information

Document tabled:

Upcoming Q3/2017 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/