5 December 2016
EMA/COMP/734740/2016
Procedure Management and Committees Support Division

Committee for Orphan Medicinal Products (COMP)

Draft agenda for the meeting on 06-08 December 2016

Chair: Bruno Sepodes – Vice-Chair: Lesley Greene

06 December 2016, 09:00-19:00, room 2F

07 December 2016, 08:30-19:00, room 2F

08 December 2016, 08:30-15:30, 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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Table of contents

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 06-08 December 2016. See December 2016 COMP minutes (to be published post January 2016 COMP meeting).

1.2. Adoption of agenda

COMP agenda for 06-08 December 2016.

1.3. Adoption of the minutes

COMP minutes for 03-04 November 2016.

2. Applications for orphan medicinal product designation

2.1. For opinion

2.1.1. - EMA/OD/200/16

Treatment of paediatric stroke

Action: For adoption, Oral explanation to be held on 06 December 2016 at 09:30

Documents tabled:
Draft Summary report with response to LoQs

2.1.2. - EMA/OD/205/16

Treatment of Cockayne syndrome

Action: For adoption, Oral explanation to be held on 06 December 2016 at 11:00

Documents tabled:
Draft Summary report with response to LoQs

2.1.3. - EMA/OD/203/16

Treatment of Rett syndrome

Action: For adoption, Oral explanation to be held on 06 December 2016 at 12:00

Documents tabled:
Draft Summary report with response to LoQs

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

2.1.4. - EMA/OD/210/16

Prevention of necrotising enterocolitis

Action: For adoption, Oral explanation to be held on 06 December 2016 at 14:30

Documents tabled:
Draft Summary report with response to LoQs

Notes:
There have been 2 designations for this condition: EMA/OD/112/13 Lactobacillus acidophilus / Bifidobacterium bifidum, EMA/OD/237/14 Lactobacillus reuteri

2.1.5. - EMA/OD/215/16

Treatment of glioma

Action: For adoption, Oral explanation to be held on 06 December 2016 at 15:30

Documents tabled:
Draft Summary report with response to LoQs

Notes:
There have been 42 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 limpet haemocyanin, 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

Designations withdrawn: EMEA/OD/067/01 Carmustine (solution for intratumoral injection), 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/037/02 Iodine (131I) anti-nucleohistone H1 chimeric biotinylated monoclonal antibody, EMEA/OD/067/03 Cilengitide, EMEA/OD/050/06 Iodine (131I) anti-tenascin monoclonal antibody 81C6, EMEA/OD/112/08 Talampanel, EMEA/OD/004/09 4,6,8-trihydroxy-10-(3,7,11-trimethyldodeca-2,6,10-trienyl)-5,10-dihydrodibenzo[b,e][1,4] diazepin-11-one, EMA/OD/031/10 Glutathione-pegylated liposomal doxorubicin hydrochloride, EMA/OD/049/12 Humanised monoclonal antibody against epidermal growth factor receptor, EMA/OD/113/15 Dronabinol and cannabidiol

2.1.6. - EMA/OD/214/16

Treatment of status epilepticus

Action: For adoption, Oral explanation to be held on 06 December 2016 at 17:00

Documents tabled:
Draft Summary report with response to LoQs

2.1.7. - EMA/OD/199/16

Treatment of pancreatic cancer

Action: For adoption, Oral explanation to be held on 07 December 2016 at 09:00

Documents tabled:
Draft Summary report with response to LoQs

Notes:
There have been 33 designations for this condition: EMEA/OD/055/02 G17(9) gastrin-Diphtheria Toxoid Conjugate, EMEA/OD/068/02 Rubitecan, EMA/OD/302/14 Human reovirus type 3 Dearing strain, 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 (TEXT TOO LONG), 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/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

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

2.1.8. - EMA/OD/196/16

Treatment of Guillain-Barré syndrome

Action: For information

Documents tabled:
Withdrawal request of 20 November 2016

Notes:
There has been 1 designation for this condition: EMA/OD/030/16 Recombinant protein derived from the saliva of the Ornithodoros moubata tick

Designation withdrawn: EMEA/OD/101/06 Fampridine

2.1.9. - EMA/OD/189/16

Treatment of acute sensorineural hearing loss

Action: For adoption, Oral explanation to be held on 07 December 2016 at 11:00

Documents tabled:
Draft Summary report with response to LoQs

2.1.10. - EMA/OD/192/16

Treatment of pulmonary arterial hypertension

Action: For adoption, Oral explanation to be held on 07 December 2016 at 12:00

Documents tabled:
Draft Summary report with response to LoQs

Notes:
There have been 4 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

2.1.11. - EMA/OD/097/16

Treatment of primary hypogonadotropic hypogonadism

Action: For adoption, Oral explanation to be held on 07 December 2016 at 14:30

Documents tabled:
Draft Summary report with response to LoQs

2.1.12. - EMA/OD/213/16

Treatment of antiphospholipid syndrome

Action: For adoption

Documents tabled:
Draft Summary report with response to LoQs

2.1.13. - EMA/OD/212/16

Treatment of multiple myeloma

Action: For adoption, Oral explanation to be held on 07 December 2016 at 18:00

Documents tabled:
Draft Summary report with response to LoQs

Notes:
There have been 14 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

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. For discussion / preparation for an opinion

2.2.1. - EMA/OD/243/16

Treatment of systemic sclerosis

Action: For adoption

Documents tabled:
Draft Summary report

Notes:
There have been 15 designations for this condition: EMEA/OD/032/01 Halofuginone hydrobromide, EMEA/OD/035/05 Peptide 144 TGF-beta1-inhibitor (TSLDASIIWAMMQN), EMEA/OD/079/08 Type I native bovine skin collagen, EMEA/OD/106/08 Treprostinil diethanolamine, EMA/OD/095/10 Paquinimod, EMA/OD/143/12 2-[4-Methoxy-3-(2-m-tolyl-ethoxy)-benzoylamino]-indan-2-carboxylic acid, EMA/OD/153/12 Terguride, EMA/OD/044/14 Riociguat, EMA/OD/129/14 1-(6-benzothiazolylsulfonyl)-5-chloro-1H-indole-2-butanoic acid, EMA/OD/148/14 Humanized IgG1 monoclonal antibody against human eotaxin-2, EMA/OD/225/14 Nitroglycerin, EMA/OD/296/14 Autologous adipose tissue-derived stromal vascular fraction cells, EMA/OD/105/15 2-(2-chlorophenyl)-4-[3-(dimethylamino)phenyl]-5-methyl-1H-pyrazolo[4,3-C]pyridine-3,6(2H,5H)-dione, EMA/OD/257/15 Autologous stromal vascular cell fraction from adipose tissue, EMA/OD/095/16 Nintedanib

Designations withdrawn: EMEA/OD/051/01 Human engineered monoclonal antibody specific for Transforming Growth Factor ß1, EMA/OD/163/11 Pomalidomide, EMA/OD/156/12 Terguride

2.2.2. - EMA/OD/231/16

Treatment of Alström syndrome

Action: For adoption

Documents tabled:
Draft Summary report

2.2.3. - EMA/OD/229/16

Treatment of diffuse large B cell lymphoma

Action: For adoption

Documents tabled:
Draft Summary report

Notes:
There have been 3 designations for this condition: EMEA/OD/097/06 Enzastaurin hydrochloride, EMA/OD/071/14 (Z)-3-(3-(3,5-bis(trifluoromethyl)phenyl)-1H-1,2,4-triazol-1-yl)-N'-(pyrazin-2-yl)acrylohydrazide, EMA/OD/171/14 Autologous T cells transduced with retroviral vector encoding an anti-CD19 CD28/CD3 zeta chimeric antigen receptor

2.2.4. - EMA/OD/080/15

Treatment of fragile X syndrome

Action: For adoption

Documents tabled:
Draft Summary report

Notes:
There have been 7 designations for this condition: EMA/OD/144/10 R-baclofen, EMA/OD/059/12 Mavoglurant, EMA/OD/105/14 (3S)-(+)-(5-chloro-2-methoxyphenyl)-1,3-dihydro-3-fluoro-6-(trifluoromethyl)-2H-indol-2-one, EMA/OD/137/14 Acamprosate calcium, EMA/OD/253/14 Tideglusib, EMA/OD/055/15 Glycyl-L-2-methylprolyl-L-glutamic acid, EMA/OD/034/16 Pyridoxine and L-pyroglutamic acid

2.2.5. - EMA/OD/241/16

Treatment of pancreatic cancer

Action: For adoption

Documents tabled:
Draft Summary report

Notes:
There have been 33 designations for this condition: Please see 2.1.7.

2.2.6. - EMA/OD/238/16

Treatment of haemophilia A

Action: For adoption

Documents tabled:

Draft Summary report

Notes:
There have been 10 designations for this condition: EMA/OD/128/10 Pegylated B-domain-deleted sequence-modified recombinant human factor VIII, EMA/OD/132/10 Recombinant fusion protein linking human coagulation factor VIIa with human albumin (rVIIa-FP), EMA/OD/144/11 Pegylated recombinant factor VIII, EMA/OD/095/12 Humanised monoclonal IgG4 antibody against tissue factor pathway inhibitor, EMA/OD/039/14 Synthetic double-stranded siRNA oligonucleotide directed against antithrombin mRNA that is covalently linked to a ligand containing three N-acetylgalactosamine residues, EMA/OD/144/13 Humanised monoclonal modified IgG4 antibody with bispecific structure targeting factors IX, IXa, X and Xa, EMA/OD/069/14 Recombinant factor VIIa modified with three terminal repeats derived from the β chain of human chorionic gonadotropin, EMA/OD/123/14 A combination of H-Lys-Lys-Gly-Pro-Arg-Cys(SH)-Leu-Thr-Arg-Tyr-Tyr-Ser-Ser-Phe-Val-Asn-Met-Glu-Gly-Lys-Lys-OH and H-Lys-Lys-Gly-Asp-Asn-Ile-Met-Val-Thr-Phe-Arg-Asn-Gln-Ala-Ser-Arg-Pro-Tyr-Gly-Lys-Lys-OH, EMA/OD/230/15 adeno-associated viral vector serotype 5 containing a B-domain deleted variant of human coagulation factor VIII gene, EMA/OD/093/16 Human monoclonal IgG1 antibody against tissue factor pathway inhibitor

Designations withdrawn: EMEA/OD/031/09 Sequence-modified recombinant human factor VIIa, EMA/OD/030/10 Recombinant fusion protein consisting of human coagulation factor VIII attached to the Fc domain of human IgG1, EMA/OD/043/10 Recombinant porcine factor VIII (B domain deleted), EMA/OD/069/12 vatreptacog alfa (activated)

2.2.7. - EMA/OD/211/16

Treatment of glioma

Action: For adoption

Documents tabled:
Draft Summary report

Notes:
There have been 42 designations for this condition: Please see 2.1.5.

2.2.8. - EMA/OD/235/16

Treatment of perinatal intracranial haemorrhage

Action: For adoption

Documents tabled:
Draft Summary report

2.2.9. - EMA/OD/216/16

Treatment of ovarian cancer

Action: For adoption

Documents tabled:
Draft Summary report

Notes:
There have been 30 designations for this condition: EMEA/OD/019/02 Oregovomab, EMEA/OD/080/03 Anti-epithelial cell adhesion molecule/anti-CD3 monoclonal antibody, EMEA/OD/044/03 Trabectedin, EMEA/OD/061/06 Paclitaxel (micellar), EMA/OD/304/14 Human reovirus type 3 Dearing strain, EMA/OD/314/14 {2-amino-8-[4-(pyrrolidinylcarbonyl)phenyl]-(3H-benzo[f]azepin-4-yl)}-N,N-dipropylcarboxamide, EMEA/OD/065/05 Imexon, EMEA/OD/063/07 Olaparib, EMEA/OD/110/07 Humanised monoclonal antibody to the folate receptor alpha, EMEA/OD/006/09 Human MHC non-restricted cytotoxic T-cell line, EMEA/OD/086/09 8-[4-(1-aminocyclobutyl)phenyl]-9-phenyl-1,2,4-triazolo[3,4-f][1,6]naphthyridin-3(2H)-one mono-hydrochloride, EMA/OD/015/10 (3S)-3-{4-[7-(aminocarbonyl)-2H-indazol-2-yl] phenyl} piperidine tosylate monohydrate salt, EMA/OD/021/10 Autologous dendritic cells pulsed with recombinant human-fusion protein (mucin 1 - glutathione S transferase) coupled to oxidised polymannose, EMA/OD/014/10 Pyr-His-Trp-Ser-Tyr-D-Lys(doxorubicinylglutarate)-Leu-Arg-Pro-Gly-NH2, acetate salt, EMA/OD/111/10 Veliparib, EMA/OD/054/11 20-pentaerythritol poly (oxy-1,2-ethanediyl)-carboxymethyl-glycinate-7-ethyl-10-hydroxycamptothecine 10-[1,4'-bipiperidine]-1'-carboxylate, EMA/OD/151/11 2-Allyl-1-[6-(1-hydroxy-1-methylethyl)pyridin-2-yl]-6-{[4-(4-methylpiperazin-1-yl)phenyl]amino}-1,2-dihydro-3H-pyrazolo[3,4-d]pyrimidin-3-one, EMA/OD/085/12 rucaparib, EMA/OD/099/12 Lurbinectedin, EMA/OD/114/12 Alisertib, EMA/OD/147/12 Chimeric monoclonal antibody against claudin 6, EMA/OD/039/13 Fosbretabulin tromethamine, EMA/OD/122/13 Trebananib, EMA/OD/186/13 Genetically modified serotype 5/3 adenovirus coding for granulocyte macrophage colony-stimulating factor, EMA/OD/059/14 Cediranib, EMA/OD/281/14 Humanised anti-folate receptor 1 monoclonal antibody conjugated to maytansinoid DM4, EMA/OD/157/14 2-hydroxymethyl-2-methoxymethyl-1-azabicyclo[2,2,2]octan-3-one, EMA/OD/211/14 Chimeric group B adenovirus (11p/3) with deletions in the E3 and E4 regions, EMA/OD/223/14 N-methyl-4-({4-[({3-methyl(methylsulfonyl)amino]pyrazin-2-yl}methyl)amino]-5- (trifluoromethyl)pyrimidin-2-yl}amino)benzamide hydrochloride, EMA/OD/126/15 (5S,8S,10aR)-N-benzhydryl-5-((S)-2-(methylamino)propanamido)-3-(3-methylbutanoyl)-6-oxodecahydropyrrolo[1,2-a][1,5]diazocine-8-carboxamide

Designations withdrawn: EMEA/OD/061/00 Human Milk Fat Globule 1 / Yttrium (90Y) human Milk Fat Globule 1 - S p isothiocyanatobenzyl-diethylenetriaminepentaacetic acid, EMEA/OD/062/01 Epothilone B, EMEA/OD/016/03 Murine anti-idiotypic antibody against OC125 antibody against CA125 antigen, EMEA/OD/071/09 Anti-EphA2 monoclonal antibody conjugated to maleimidocaproyl monomethylauristatin phenylalanine, EMA/OD/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

2.2.10. - EMA/OD/246/16

Treatment of non-infectious uveitis

Action: For adoption

Documents tabled:
Draft Summary report

Notes:
There have been 6 designations for this condition: EMA/OD/118/12 Voclosporin, EMA/OD/024/15 3-{[2,3,5,6-tetrafluoro-3'-(trifluoromethoxy)biphenyl-4-yl]carbamoyl}thiophene-2-carboxylic acid, EMA/OD/195/14 Autologous collagen type II-specific regulatory T cells, EMA/OD/320/14 Triamcinolone acetonide, EMA/OD/207/15 DNA plasmid encoding a recombinant fusion protein consisting of the extracellular domain of human TNFα p55 receptor linked to the human IgG1 Fc domain, EMA/OD/219/15 Fluocinolone acetonide

2.2.11. - EMA/OD/244/16

Treatment of primary ciliary dyskinesia

Action: For adoption

Documents tabled:
Draft Summary report

2.2.12. - EMA/OD/232/16

Treatment of glycogen storage disease type II (Pompe's disease)

Action: For adoption

Documents tabled:
Draft Summary report

Notes:
There have been 2 designations for this condition: EMA/OD/057/11 Glycosylation independent lysosomal targeting tagged recombinant human acid alpha glucosidase, EMA/OD/098/16 Recombinant human acid alpha-glucosidase conjugated with mannose-6-phosphate analogues

2.2.13. - EMA/OD/250/16

Treatment of malignant mesothelioma

Action: For adoption

Documents tabled:
Draft Summary report

Notes:
There have been 10 designations for this condition: EMEA/OD/003/08 NGR-human Tumour Necrosis Factor, EMA/OD/063/12 Maytansinoid-conjugated human monoclonal antibody against mesothelin, EMA/OD/012/13 N-methyl-4-({4-[({3 methyl (methylsulfonyl) aminopyrazin-2-yl}methyl)amino]-5-(trifluoromethyl)pyrimidin-2-yl}amino)benzamide hydrochloride, EMA/OD/138/13 Autologous dendritic cells pulsed with allogeneic tumour cell lysate, EMA/OD/108/13 Amatuximab, EMA/OD/076/14 Pegylated recombinant arginine deiminase, EMA/OD/180/14 Genetically modified serotype 5/3 adenovirus coding for granulocyte macrophage colony-stimulating factor, EMA/OD/157/15 Live attenuated Listeria monocytogenes delta actA/delta inlB strain expressing human mesothelin, EMA/OD/243/15 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…., EMA/OD/101/16 Cisplatin

Designations withdrawn: EMEA/OD/022/01 Pemetrexed disodium, EMA/OD/028/10 Vorinostat, EMA/OD/168/14 5-[8-methyl-9-(1-methylethyl)-2-(4-morpholinyl)-9H-purin-6-yl]-2-pyrimidinamine

2.2.14. - EMA/OD/233/16

Treatment of Lennox-Gastaut syndrome

Action: For adoption

Documents tabled:
Draft Summary report

Notes:
There has been 1 designation for this condition: EMEA/OD/047/04 Rufinamide

2.2.15. - EMA/OD/239/16

Treatment of pancreatic cancer

Action: For adoption

Documents tabled:
Draft Summary report

Notes:
There have been 33 designations for this condition: Please see 2.1.7.

2.2.16. - EMA/OD/230/16

Treatment of eosinophilic oesophagitis

Action: For adoption

Documents tabled:
Draft Summary report

Notes:
There have been 3 designations for this condition: EMA/OD/078/13 Budesonide, EMA/OD/118/13 Human monoclonal antibody against human interleukin 13, EMA/OD/004/16 Humanised monoclonal antibody targeting interleukin-15

2.2.17. - EMA/OD/194/16

Treatment of oculopharyngeal muscular dystrophy

Action: For adoption

Documents tabled:
Draft Summary report

Notes:
There has been 1 designation for this condition: EMA/OD/008/15 Trehalose

2.2.18. - EMA/OD/251/16

Treatment of sickle cell disease

Action: For adoption

Documents tabled:
Draft Summary report

Notes:
There have been 10 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

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

2.2.19. - EMA/OD/191/16

Treatment of haematopoietic stem and progenitor-cell transplant

Action: For adoption

Documents tabled:
Draft Summary report

2.2.20. - EMA/OD/222/16

Treatment of acute liver failure

Action: For adoption

Documents tabled:
Draft Summary report

Notes:
There have been 7 designations for this condition: EMEA/OD/037/05 Human heterologous liver cells (for infusion), EMEA/OD/085/08 Recombinant human hepatocarcinoma-intestine-pancreas / pancreatic associated protein, EMA/OD/030/11 Cardiotrophin-1, EMA/OD/105/11 Ornithine phenylacetate, EMA/OD/153/11 Heterologous human adult liver-derived stem cells, EMA/OD/032/13 Immortalised human C3A hepatoblastoma cells, EMA/OD/022/16 Citric acid monohydrate

2.2.21. - EMA/OD/240/16

Treatment of medulloblastoma

Action: For adoption

Documents tabled:
Draft Summary report

Notes:
There has been 1 designation for this condition: EMA/OD/020/10 16-base single-stranded peptide nucleic acid oligonucleotide linked to a 7-amino acid peptide

2.2.22. - EMA/OD/252/16

Treatment of acetaminophen (paracetamol) overdose

Action: For adoption

Documents tabled:
Draft Summary report

2.2.23. - EMA/OD/228/16

Treatment of gastric cancer

Action: For adoption

Documents tabled:
Draft Summary report

Notes:
There have been 5 designations for this condition: EMEA/OD/056/02 G17(9) gastrin-Diphtheria Toxoid Conjugate, EMEA/OD/044/06 Catumaxomab, EMA/OD/083/10 Chimeric monoclonal antibody against claudin-18 splice variant 2, EMA/OD/101/10 Tesetaxel, EMA/OD/012/14 Rilotumumab

Designations withdrawn: EMEA/OD/073/07 Tegafur, gimeracil, oteracil potassium, EMA/OD/022/11 Everolimus, EMA/OD/030/12 Ramucirumab

2.2.24. - EMA/OD/130/16

Treatment of interstitial cystitis

Action: For adoption

Documents tabled:
Draft Summary report

Notes:
There have been 2 designations for this condition: EMA/OD/179/14 Pentosan polysulfate sodium, EMA/OD/003/16 Pentosan polysulfate sodium

2.2.25. - EMA/OD/220/16

Treatment of perinatal asphyxia

Action: For adoption

Documents tabled:

Draft Summary report

Notes:
There have been 6 designations for this condition: EMEA/OD/109/09 2-iminobiotin, EMA/OD/133/11 Melatonin, EMA/OD/134/12 Allopurinol sodium, EMA/OD/315/14 Xenon, EMA/OD/004/15 Allopurinol sodium, EMA/OD/042/15 Cannabidiol

2.2.26. - EMA/OD/226/16

Treatment of mucopolysaccharidosis type IIIB (Sanfilippo B syndrome)

Action: For adoption

Documents tabled:
Draft Summary report

Notes:
There have been 3 designations for this condition: EMA/OD/065/11 Adeno-associated viral vector containing the human alpha-N-acetylglucosaminidase gene, EMA/OD/150/12 Adeno-associated viral vector serotype 9 containing the human N-acetylglucosaminidase alpha gene, EMA/OD/035/13 Recombinant human alpha-N-acetylglucosaminidase

2.2.27. - EMA/OD/247/16

Treatment of bronchiolitis obliterans syndrome

Action: For adoption

Documents tabled:
Draft Summary report

2.2.28. - EMA/OD/249/16

Treatment of glioma

Action: For adoption

Documents tabled:
Draft Summary report

Notes:
There have been 42 designations for this condition: Please see 2.1.5.

2.2.29. - EMA/OD/237/16

Prevention of graft rejection following solid organ transplantation

Action: For adoption

Documents tabled:
Draft Summary report

Notes:
There have been 5 designations for this condition: EMA/OD/308/14 Recombinant monoclonal IgG1 antibody against T-cell immune response cDNA 7, EMA/OD/165/12 Murine

IgM monoclonal antibody binding to alpha beta T-Cell receptor, EMA/OD/043/13 Autologous regulatory T cells with an immunophenotype of CD4+CD25hiFoxP3+, EMA/OD/176/13 Eculizumab, EMA/OD/168/13 Ex vivo cultured human mesenchymal stromal cells

2.2.30. - EMA/OD/224/16

Treatment of cystic fibrosis

Action: For adoption

Documents tabled:
Draft Summary report

Notes:
There have been 37 designations for this condition: EMEA/OD/011/03 Recombinant dog gastric lipase, EMEA/OD/032/00 L-Lysine-N-Acetyl-L-Cysteinate, 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/100/16 (6aR, 10aR)-3-(1',1'-dimethylheptyl)-delta-8-tetrahydro-cannabinol-9-carboxylic acid

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

2.2.31. - EMA/OD/245/16

Treatment of ovarian cancer

Action: For adoption

Documents tabled:
Draft Summary report

Notes:
There have been 30 designations for this condition: Please see 2.2.9.

2.2.32. - EMA/OD/221/16

Treatment of Dravet syndrome

Action: For adoption

Documents tabled:
Draft Summary report

Notes:
There have been 2 designations for this condition: EMA/OD/140/13 Fenfluramine hydrochloride, EMA/OD/083/14 Cannabidiol

2.2.33. - EMA/OD/209/16

Treatment of spinocerebellar ataxia

Action: For adoption

Documents tabled:
Draft Summary report

Notes:
There have been 2 designations for this condition: EMA/OD/216/14 Ceftriaxone, EMA/OD/009/15 Trehalose

2.3. Amendment of existing orphan designations

2.3.1. Synthetic double-stranded siRNA oligonucleotide directed against transthyretin Mrna – EMA/OD/142/10, EU/3/11/857

Alnylam UK Limited - United Kingdom; Treatment of familial amyloid polyneuropathy;

Action: For adoption

Document tabled:

Amended draft Summary report

2.4. COMP opinions adopted via written procedure following previous meeting

2.4.1. 20% intravenous fat emulsion consisting of 20% soybean oil, 1.2% egg yolk phospholipids, 2.25% glycerin, and water for injection - EMA/OD/062/16

Alan Boyd Consultants Ltd; Treatment of poisoning by local anaesthetics

COMP coordinator: Violeta Stoyanova

Action: For information

Document tabled:
Summary report

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 06-08 December 2016 COMP meeting

2.7. Evaluation on-going

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

Action: For information

Notes:
See 6.8.1. Table 6. Evaluation Ongoing.

3. Requests for protocol assistance with significant benefit question

3.1. Ongoing procedures

None

3.2. Finalised letters

3.2.1. -

Treatment in haematopoietic stem cell transplantation

Action: For information

3.3. New requests

3.3.1. -

Treatment of paroxysmal nocturnal haemoglobinuria

Action: For information

3.3.2. -

Treatment of autosomal dominant polycystic kidney disease

Action: For information

3.3.3. -

Treatment of glioma

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. Cystadrops (mercaptamine) - EMA/OD/036/08, EU/3/08/578, EMEA/H/C/003769

Orphan Europe S.A.R.L.; Treatment of cystinosis

Action: For adoption

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

Notes:
Status of the procedure at the CHMP: Opinion adopted in October 2016

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

4.2.1. - chlormethine –EMA/OD/112/11, EU/3/12/963, EMEA/H/C/002826

Actelion Registration Ltd.; Treatment of cutaneous T-cell lymphoma

Action: For discussion

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

4.3. On-going procedures

Action: For information

4.4. Public Summary of Opinion

None

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

None

6. Organisational, regulatory and methodological matters

6.1. Mandate and organisation of the COMP

6.1.1. Strategic Review & Learning meetings

COMP Strategy Review & Learning meetings, 20-21 March 2016, Valletta, Malta

Action: For information

Document tabled:
Draft Agenda awaited

6.1.2. Protocol Assistance Working Group

Proposed meeting time on 08 December 2016 at time 08:30, room 2H (To be confirmed)

Document(s) tabled:
Awaited

6.1.3. COMP Drafting Group

Proposed meeting time on 07 December 2016 at time 08:30, room 2H (To be confirmed)

6.1.4. Preclinical Models Working Group

Proposed meeting time on 08 December 2016 at time 14:30, room 2J (To be confirmed)

6.1.5. Recommendations on eligibility to PRIME – report from CHMP

Action: For information

Document(s) tabled:
PRIME eligibility requests - list of adopted outcomes October 2016

6.1.6. Updated policy on handling competing interests for scientific committees' members and experts

Action: For information

Document(s) tabled:
Policy 0044 - European Medicines Agency policy on the handling of competing interests of scientific committees' members and experts

6.2. Coordination with EMA Scientific Committees or CMDh-v

6.2.1. PDCO/COMP Working Group

Proposed meeting time on 07 December 2016 at time 13:00 (To be confirmed)

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

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

None

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

None

6.4. Cooperation within the EU regulatory network

6.4.1. European Commission

Commission Notice on the application of Articles 3, 5 and 7 of Regulation (EC) No 141/2000 on Orphan Medicinal Products

Action: For information

6.5. Cooperation with International Regulators

6.5.1. Food and Drug Administration (FDA)

Action: For information

Document tabled:
Draft Agenda November 8, 2016

6.5.2. Gaucher disease - A Strategic Collaborative Approach from EMA and FDA

Action: For information

Document tabled:
Awaited

6.5.3. Japanese Pharmaceuticals and Medical Devices Agency (PMDA)

None

6.5.4. The Therapeutic Goods Administration (TGA), Australia

None

6.5.5. Health Canada (HC)

None

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

None

6.7. COMP work plan

6.7.1. COMP Work Plan 2016

Action: For information

Document(s) tabled:
COMP Work Plan 2016
COMP Work plan tracking tool 2016

6.7.2. COMP Work Plan 2017

Action: For adoption

Document(s) tabled:
COMP draft Work Plan 2017

6.8. Planning and reporting

6.8.1. List of all applications submitted/expected and the COMP coordinatorship distribution of valid applications submitted in 2016

Action: For information

6.8.2. Overview of orphan marketing authorisations/applications

Action: For information

7. Any other business

7.1. EMA Business Pipeline activity and Horizon scanning

Action: For information

Document tabled:
Awaited

8. Explanatory notes

The notes below give a brief explanation of the main sections and headings in the COMP agenda and should be read in conjunction with the agenda or the minutes.

Abbreviations / Acronyms

CHMP: Committee for Medicinal Product for Human Use
COMP: Committee for Orphan Medicinal Products
EC: European Commission
FAL: Final Advice Letter
OD: Orphan Designation
OMPD: Orphan Medicinal Product 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/