6 July 2016
EMA/COMP/428333/2016
Procedure Management and Committees Support Division

Committee for Orphan Medicinal Products (COMP)

Draft agenda for the meeting on 11-13 July 2016

Chair: Bruno Sepodes – Vice-Chair: Lesley Greene

11 July 2016, 09:00-19:00, room 2F

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

13 July 2016, 08:30-16:00, 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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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 11-13 July 2016. See July 2016 COMP minutes (to be published post September 2016 COMP meeting).

1.2. Adoption of agenda

COMP agenda for 11-13 July 2016.

1.3. Adoption of the minutes

COMP minutes for 14-16 June 2016.

2. Applications for orphan medicinal product designation

2.1. For opinion

2.1.1. - EMA/OD/073/16

Treatment of McArdle disease

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

Documents tabled:
Draft Summary report with response to LoQs

2.1.2. - EMA/OD/059/16

Treatment of argininosuccinic aciduria

Action: For adoption, Oral explanation to be held on 11 July 2016 at time 12:00

Documents tabled:
Draft Summary report with response to LoQs

Notes:
There have been 4 designations for this condition: EMA/OD/004/10 glyceryl tri-(4-phenybutyrate), EMA/OD/107/10 Human heterologous liver cells (for infusion), EMA/OD/059/13 Heterologous human adult liver-derived progenitor cells, EMA/OD/124/15 Sodium benzoate

2.1.3. - EMA/OD/056/16

Treatment of ornithine translocase deficiency

Action: For adoption, Oral explanation to be held on 11 July 2016 at time 12:00

Documents tabled:

Draft Summary report with response to LoQs

Notes:
There have been 2 designations for this condition: EMA/OD/063/13 Heterologous human adult liver-derived progenitor cells, EMA/OD/006/10 Glyceryl tri-(4-phenybutyrate)

2.1.4. - EMA/OD/055/16

Treatment of lysinuric protein intolerance

Action: For adoption, Oral explanation to be held on 11 July 2016 at time 12:00

Documents tabled:
Draft Summary report with response to LoQs

2.1.5. - EMA/OD/054/16

Treatment of N-acetylglutamate synthase deficiency

Action: For adoption, Oral explanation to be held on 11 July 2016 at time 12:00

Documents tabled:
Draft Summary report with response to LoQs

Notes:
There has been 1 designation for this condition: EMA/OD/061/13 Heterologous human adult liver-derived progenitor cells

2.1.6. - EMA/OD/080/16

Treatment of graft versus host disease

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

Documents tabled:
Draft Summary report with response to LoQs

Notes:
There have been 10 designations for this condition: EMA/OD/017/16 Rimiducid, EMEA/OD/009/06 Methoxsalen, EMEA/OD/046/05 A mixture of anti-CD3 mAb (SPV-T3a)-ricin A chain fusion protein and anti-CD7 mAb (WT1)-ricin A chain fusion protein, EMEA/OD/054/06 Recombinant fusion protein consisting of the extracellular portion of CD95 fused to the Fc part of a human IgG1 molecule, EMEA/OD/049/06 Budesonide, EMEA/OD/068/06 Ex-vivo cultured adult human mesenchymal stem cells, EMEA/OD/038/00 Inolimomab, EMEA/OD/036/01 beclomethasone 17, 21-dipropionate (oral use), EMA/OD/248/15 Cannabidiol, EMA/OD/017/16 Rimiducid

Designations withdrawn: EMEA/OD/045/02 anti-CD 147 murine monoclonal IgM, EMEA/OD/020/00 Thalidomide

2.1.7. - EMA/OD/078/16

Treatment of idiopathic dilated cardiomyopathy

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

Documents tabled:
Draft Summary report with response to LoQs

2.1.8. - EMA/OD/077/16

Treatment of paroxysmal nocturnal haemoglobinuria

Action: For adoption, Oral explanation to be held on 12 July 2016 at time 09:30

Documents tabled:
Draft Summary report with response to LoQs

Notes:
There have been 3 designations for this condition: EMEA/OD/042/03 Eculizumab, EMA/OD/098/14 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) , EMA/OD/246/15 Fc- and CDR-modified humanised monoclonal antibody against C5

Designation withdrawn: EMEA/OD/016/02 Myristolated-peptidyl-recombinant Human CD59

2.1.9. - EMA/OD/050/16

Treatment of Huntington's disease

Action: For adoption, Oral explanation to be held on 12 July 2016 at time 11:00

Documents tabled:
Draft Summary report with response to LoQs

Notes:
There have been 6 designations for this condition: EMA/OD/070/14 Cysteamine bitartrate , EMA/OD/169/14 5-bromo-N-(prop-2-yn-1-yl)-2-(1H-1,2,4-triazol-1-yl) pyrimidine-4,6-diamine , EMA/OD/256/14 Chimeric 2'-O-(2-methoxyethyl) modified oligonucleotide targeted to huntingtin RNA , EMA/OD/311/14 Phenol, 4-[2-(aminomethyl)-4-thiazolyl]-2,6-bis(1,1-dimethylethyl) monohydrochloride , EMA/OD/325/14 AASSGVSTPGSAGHDIITEQPRS , EMA/OD/017/15 5,7-dichloro-2-dimethylaminomethyl-8-hydroxyquinoline hydrochloride

2.1.10. - EMA/OD/051/16

Treatment of West Nile virus infection

Action: For information

Documents tabled:
Withdrawal request of 23 June 2016

2.1.11. - EMA/OD/079/16

Treatment of narcolepsy

Action: For adoption, Oral explanation to be held on 12 July 2016 at time 14:30

Documents tabled:
Draft Summary report with response to LoQs

Notes:
There have been 3 designations for this condition: EMEA/OD/087/06 1-{3-[3-(4-chlorophenyl)propoxy]propyl}piperidine, hydrochloride, EMA/OD/254/14 Mazindol, EMA/OD/002/15 Mazindol

Designation withdrawn: EMEA/OD/051/02 Sodium oxybate

2.1.12. - EMA/OD/081/16

Treatment of amyotrophic lateral sclerosis

Action: For adoption, Oral explanation to be held on 12 July 2016 at time 15:30

Documents tabled:
Draft Summary report with response to LoQs

Notes:
There have been 16 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

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

2.1.13. - EMA/OD/069/16

Treatment of tracheal stenosis

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

Documents tabled:
Draft Summary report with response to LoQs

2.1.14. - EMA/OD/067/16

Treatment of glioma

Action: For adoption

Documents tabled:
Draft Summary report with response to LoQs

Notes:

There have been 41 designations for this condition: EMEA/OD/004/02 Pseudomonas exotoxin (domains II/III)-Interleukin 13 chimeric protein, EMEA/OD/037/02 Iodine (131I) anti-nucleohistone H1 chimeric biotinylated monoclonal antibody, EMEA/OD/026/03 Herpes simplex virus lacking infected cell protein 34.5, EMEA/OD/055/03 Gimatecan, EMEA/OD/050/04 Biotinylated anti-tenascin monoclonal antibody for use with 90-Yttrium, EMEA/OD/038/04 Anti epidermal growth factor receptor antibody h-R3, EMEA/OD/030/05 Oligonucleotide phosphorothioate (TAAACGTTATAACGTTATGACGTCAT), sodium salt, EMEA/OD/068/05 Enzastaurin hydrochloride, EMEA/OD/110/05 4-[131I] iodo-L-phenylalanine, EMEA/OD/081/06 Autologous dendritic cells pulsed with autologous tumour cell lysate, EMEA/OD/050/07 Doxorubicin hydrochloride (drug eluting beads), EMEA/OD/051/07 Irinotecan hydrochloride (drug eluting beads), EMEA/OD/038/07 Iodine (131I) Chlorotoxin, EMEA/OD/004/08 Recombinant fusion protein of circulary-permuted IL-4 and pseudomonas exotoxin A, [IL-4(38-37)-PE38KDEL], EMEA/OD/023/08 Topotecan hydrochloride (liposomal), EMEA/OD/034/08 Gadodiamide (liposomal), EMEA/OD/104/08 Autologous tumour-derived gp96 heat shock protein-peptide complex, EMEA/OD/098/09 Recombinant fusion protein consisting of the extracellular portion of CD95 fused to the Fc part of a human IgG1 molecule, EMA/OD/086/10 7-beta-hydroxycholesteryl-3-beta-oleate, EMA/OD/092/12 IL-12-secreting dendritic cells, loaded with autologous tumour lysate , EMA/OD/077/11 L-cysteine, L-leucyl-L-alpha-glutamyl-L-alpha-glutamyl-L-lysyl-L-lysylglycyl-L-asparaginyl-L-tyrosyl-L-valyl-L-valyl-L-threonyl-L-alpha-aspartyl-L-histidyl-S-[1-[(4-carboxycyclohexyl)methyl]-2,5-dioxo-3-pyrrolidinyl]-complex with keyhole limdinator_Applica, EMA/OD/050/11 2-hydroxyoleic acid, EMA/OD/157/11 Adenovirus-associated vector containing human Fas-c gene , EMA/OD/019/12 Doxorubicin (administered after synthetic double-stranded siRNA oligonucleotide directed against claudin-5 complexed with polyethyleneimine) , EMA/OD/170/12 4-[2-(6-methylpyridin-2-yl)-5,6-dihydro-4H-pyrrolo[1,2-b]pyrazol-3-yl]-quinoline-6-carboxamide monohydrate , EMA/OD/148/12 1,2:5,6-Dianhydrogalactitol , EMA/OD/136/12 Synthetic double-stranded siRNA oligonucleotide directed against Claudin-5 complexed with polyethyleneimine (prior to administration of doxorubicin) , EMA/OD/086/13 Autologous ex vivo expanded leukocytes treated with 5-aza-2'-deoxycytidine , EMA/OD/001/14 Autologous dendritic cells pulsed with RNA from glioma stem cells , EMA/OD/107/13 Allogeneic and autologous haptenised and irradiated cells and cell lysates derived from glioma , EMA/OD/174/13 Autologous dendritic cells pulsed with tumour antigen-derived synthetic peptides (MAGE-1, HER-2, AIM-2, TRP-2, gp-100, and interleukin-13 receptor alpha) , EMA/OD/111/14 Recombinant human bone morphogenetic protein 4 , EMA/OD/003/14 Paclitaxel-succinate- Arg-Arg-Leu-Ser-Tyr-Ser-Arg-Arg-Arg-Phe , EMA/OD/065/14 Humanised recombinant monoclonal antibody against epidermal growth factor receptor conjugated to maleimidocaproyl monomethylauristatin F , EMA/OD/132/14 Olaptesed pegol , EMA/OD/200/14 5,5'-(4-(trifluromethyl)benzylazanediyl)bis(methylene)diquinolin-8-ol , EMA/OD/159/14 Chloroquine, EMA/OD/176/14 Adenovirus serotype 5 containing partial E1A deletion and an integrin-binding domain , EMA/OD/251/14 Recombinant human glutamate oxaloacetate transaminase 1 , EMA/OD/206/15 N-(4-Methoxyphenyl)-N,2,6-trimethylfuro[2,3-d]pyrimidin-4-amine , EMA/OD/222/15 Delta-9-tetrahydrocannabinol and cannabidiol from extracts of the Cannabis sativa L. plant

Designations withdrawn: EMEA/OD/067/01 Carmustine (solution for intratumoral injection), EMEA/OD/074/01 Human transferrin conjugated to mutant diptheria toxin, EMEA/OD/067/03 Cilengitide, EMEA/OD/050/06 Iodine (131I) anti-tenascin monoclonal antibody 81C6, EMEA/OD/112/08 Talampanel, EMEA/OD/004/09 4,6,8-trihydroxy-10-

(3,7,11-trimethyldodeca-2,6,10-trienyl)-5,10-dihydrodibenzo[b,e][1,4] diazepin-11-one, EMA/OD/031/10 Glutathione-pegylated liposomal doxorubicin hydrochloride, EMA/OD/049/12 Humanised monoclonal antibody against epidermal growth factor receptor , EMA/OD/113/15 Dronabinol and cannabidiol

2.1.15. - EMA/OD/035/16

Treatment of fibromyalgia

Action: For adoption

Document tabled:
Summary report

Notes: Opinion was circulated for adoption via written procedure. Following comments received by COMP members, the opinion will be adopted in July.

2.2. For discussion / preparation for an opinion

2.2.1. - EMA/OD/113/16

Treatment of retinitis pigmentosa

Action: For adoption

Documents tabled:
Draft Summary report

Notes:
There have been 15 designations for this condition: EMEA/OD/057/06 4,7,10,13,16,19-Docosahexaenoic acid, EMEA/OD/043/07 Adenovirus associated viral vector serotype 4 containing the human RPE65 gene, EMEA/OD/087/08 Recombinant human proinsulin, EMA/OD/162/10 9-cis-Retinyl acetate, EMA/OD/159/11 Encapsulated human retinal pigment epithelial cell line transfected with plasmid vector expressing human ciliary neurotrophic factor , EMA/OD/006/12 Recombinant human methionine proinsulin , EMA/OD/025/13 Expanded human allogeneic neural retinal progenitor cells extracted from neural retina , EMA/OD/015/13 Recombinant human nerve growth factor , EMA/OD/031/13 Adenovirus associated viral vector serotype 5 containing the human pde6β gene , EMA/OD/289/14 Sodium 3-[(4aR,6R,7R,7aS)-7-hydroxy-2-oxido-2-sulfanylidene-4a,6,7,7a-tetrahydro-4H-furo[3,2-d][1,3,2]dioxaphosphinin-6-yl]-2-bromo-6-phenyl-5H-imidazo[1,2-a]purin-9-one , EMA/OD/271/14 Myriocin , EMA/OD/327/14 Recombinant human mesencephalic astrocyte-derived neurotrophic factor , EMA/OD/040/15 Adenovirus-associated viral vector serotype 2 containing the human RPE65 gene , EMA/OD/213/15 Allogeneic fetal human retinal progenitor cells expanded ex vivo , EMA/OD/208/15 4-[(2E)-1-oxo-3-(2,6,6-trimethyl-1-cyclohexen-1-yl)-2-propen-1-yl]-1-piperazinecarboxamide

Designations withdrawn: EMEA/OD/075/07 Recombinant human rod-derived cone viability factor, EMEA/OD/106/07 Allogeneic human umbilical cord tissue-derived cells, EMA/OD/021/12 17-(Dimethylaminoethylamino)-17-demethoxygeldanamycin (after administration of adeno-associated viral vector encoding an inducible short hairpin RNA targeting claudin-5) , EMA/OD/135/12 Adeno-associated viral vector encoding an inducible short hairpin RNA targeting claudin-5 (prior to administration of 17-

dimethylaminoethylamino-17-demethocygeldanamycin) , EMA/OD/067/13 Unoprostone isopropyl

2.2.2. - EMA/OD/100/16

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/072/05 Denufosol tetrasodium, 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

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/118/05 Glutathione, EMEA/OD/024/08 Levofloxacin hemihydrate, EMA/OD/032/14 Lumacaftor/ivacaftor

2.2.3. - EMA/OD/123/16

Treatment of osteomyelitis

Action: For adoption

Documents tabled:
Draft Summary report

2.2.4. - EMA/OD/088/16

Treatment of idiopathic pulmonary fibrosis

Action: For adoption

Documents tabled:
Draft Summary report

Notes:
There have been 12 designations for this condition: EMEA/OD/033/04 Heparin-Sodium, EMEA/OD/052/04 Pirfenidone, EMEA/OD/054/07 Interferon gamma, EMEA/OD/104/09 Macitentan, EMA/OD/079/10 2-(2-chlorophenyl)-4-[3-(dimethylamino)phenyl]-5-methyl-1H-pyrazolo[4,3-C]pyridine-3,6(2H,5H)-dione, EMA/OD/091/11 4-[[9-[(3S)-tetrahydro-3-furanyl]-8-[(2,4,6-trifluorophenyl)amino]-9H-purin-2-yl]amino]-trans-cyclohexanol, EMA/OD/048/12 Recombinant human pentraxin-2 , EMA/OD/111/12 Tralokinumab, EMA/OD/186/12 nintedanib , EMA/OD/051/14 Humanised anti-alpha v beta 6 monoclonal antibody , EMA/OD/130/14 1-(6-benzothiazolylsulfonyl)-5-chloro-1H-indole-2-butanoic acid , EMA/OD/072/15 3-pentylbenzeneacetic acid sodium salt

Designations withdrawn: EMEA/OD/002/05 Interferon gamma, EMEA/OD/075/04 Acetylcysteine, EMEA/OD/105/07 Recombinant human monoclonal antibody against transforming growth factor beta-1, 2 and 3, EMEA/OD/027/08 Bosentan, EMA/OD/029/10 Ambrisentan

2.2.5. - EMA/OD/106/16

Treatment of scedosporiosis

Action: For adoption

Documents tabled:
Draft Summary report

2.2.6. - EMA/OD/104/16

Treatment of invasive aspergillosis

Action: For adoption

Documents tabled:

Draft Summary report

Notes:
There has been 1 designation for this condition: EMA/OD/009/14 Isavuconazonium sulfate

2.2.7. - EMA/OD/119/16

Treatment of mucopolysaccharidosis type I

Action: For adoption

Documents tabled:
Draft Summary report

Notes:
There have been 3 designations for this condition: EMA/OD/121/14 Pentosan polysulfate sodium, EMA/OD/165/14 Ataluren, EMA/OD/138/14 Recombinant human insulin receptor monoclonal antibody-fused-α-L-iduronidase

2.2.8. - EMA/OD/108/16

Treatment of soft tissue sarcoma

Action: For adoption

Documents tabled:
Draft Summary report

Notes:
There have been 10 designations for this condition: EMEA/OD/001/01 Ecteinascidin 743, EMEA/OD/042/06 Doxorubicin hydrochloride (liposomal), EMA/OD/155/11 Yttrium (90Y)-DTPA-radiolabelled chimeric monoclonal antibody against frizzled homologue 10 , EMA/OD/041/13 Genetically modified serotype 5/3 adenovirus coding for granulocyte macrophage colony-stimulating factor , EMA/OD/190/13 Doxorubicin(6-maleimidocaproyl)hydrazone , EMA/OD/266/14 Olaratumab , EMA/OD/159/15 Glucopyranosyl lipid A stable emulsion and recombinant New York esophageal squamous cell carcinoma-1 protein , EMA/OD/184/15 (S)-N-(5-((R)-2-(2,5-difluorophenyl)pyrrolidin-1-yl)pyrazolo[1,5-a]pyrimidin-3-yl)-3-hydroxypyrrolidine-1-carboxamide hydrogen sulfate , EMA/OD/215/15 Human/murine chimeric monoclonal antibody against endoglin , EMA/OD/238/15 Sindbis virus envelope pseudotyped lentiviral vector encoding New York esophageal squamous cell carcinoma-1 protein

Designations withdrawn: EMEA/OD/059/03 N-acetylsarcosyl-glycyl-L-valyl-D-alloisoleucyl-L-threonyl-L-norvalyl-L-isoleucyl-L-arginyl-L-prolyl-N-ethylamide , EMEA/OD/050/05 27R,34aS)-9,27-dihydroxy-10,21-dimethoxy-6,8,12,14,20,26-hexamethyl-1,5,11,28,29-pentaoxo-1,4,5,6,9,10,11,12,13,14,21,22,23,24,25,26,27,28,29,31,32,33,34,34a-tetra-cosahydro-3H-23,27-epoxypyrido[2,1-c][1,4]oxazacyclohentriacontin-3-yl]propyl}-2-methoxy-cyclohexyldimethyl-phosphinate, EMEA/OD/071/05 Brostallicin, EMEA/OD/083/06 Fenretinide, EMEA/OD/044/08 Palifosfamide, EMA/OD/141/10 Ombrabulin, EMA/OD/110/11 (1-methyl-2-nitro-1H-imidazole-5-yl)methyl N,N'-bis(2-bromoethyl) diamidophosphate

2.2.9. - EMA/OD/096/16

Treatment of Duchenne muscular dystrophy

Action: For adoption

Documents tabled:
Draft Summary report

Notes:
There have been 24 designations for this condition: EMEA/OD/106/04 3-[5-(2-fluoro-phenyl)-[1,2,4]oxadiazole-3-yl]-benzoic acid, EMEA/OD/026/05 Adeno-associated viral vector containing a modified U7 snRNA gene, EMEA/OD/077/06 Idebenone, EMEA/OD/065/08 5-(ethylsulfonyl)-2-(naphthalen-2-yl)benzo[d]oxazole, EMEA/OD/049/08 RNA, [P-deoxy-P-(dimethylamino)] (2',3'-dideoxy-2',3'-imino-2',3'-seco) (2'a→5') (C-m5U-C-C-A-A-C-A-m5U-C-A-A-G-G-A-A-G-A-m5U-G-G-C-A-m5U-m5U-m5U-C-m5U-A-G), P-[4-[[2-[2-(2-hydroxyethoxy)ethoxy]ethoxy]carbonyl]-1-piperazinyl] N,N-dimethylam, EMEA/OD/081/08 Exon 44 specific phosphorothioate oligonucleotide, EMEA/OD/082/08 Exon 51 specific phosphorothioate oligonucleotide, EMEA/OD/044/09 Adeno-associated viral vector containing modified U1 snRNA, EMEA/OD/083/09 RNA, [P-deoxy-P-(dimethylamino)] (2',3'-dideoxy-2',3'-imino-2',3'-seco) (2'a→5') (C-m5U-m5U-A-C-A-G-G-C-m5U-C-C-A-A-m5U-A-G-m5U-G-G-m5U-C-A-G-m5U), 5' [P-[4-[[2-[2-(2-hydroxyethoxy)ethoxy]ethoxy]carbonyl]-1-piperazinyl]-N,N-dimethylaminophosphonamidate], 3'-[2'a-[N2-acetyl-L-arginyl-6-aminohexanoyl-L-arginyl-L-arginyl-β-alanyl-L-arginyl-L-arginyl-6-aminohexanoyl-L-arginyl-L-arginyl-β-alanyl-L-arginyl-6-aminohexanoyl-β-alanyl], octahydrochloride, EMA/OD/090/13 Naproxcinod, EMA/OD/142/11 Exon 45 specific phosphorothioate oligonucleotide, EMA/OD/143/11 Exon 53 specific phosphorothioate oligonucleotide , EMA/OD/162/11 Halofuginone hydrobromide , EMA/OD/028/12 Givinostat, EMA/OD/121/12 Exon 52 specific phosphorothioate oligonucleotide , EMA/OD/122/12 Exon 55 specific phosphorothioate oligonucleotide , EMA/OD/164/12 Humanised monoclonal antibody against myostatin , EMA/OD/183/12 R,S-O-(3-piperidino-2-hydroxy-1-propyl)-nicotinic acid amidoxime dihydrochloride , EMA/OD/162/13 Asp-Arg-Val-Tyr-Ile-His-Pro , EMA/OD/049/14 17α,21-dihydroxy-16α-methyl-pregna-1,4,9(11)-triene-3,20-dione , EMA/OD/166/14 Adeno-associated viral vector serotype 8 containing the human MD1 gene , EMA/OD/307/14 Rimeporide, EMA/OD/041/15 Allogeneic human adult stem cells, isolated from skeletal muscle and expanded ex vivo , EMA/OD/109/15 N-(2-((4Z,7Z,10Z,13Z,16Z,19Z)-docosa-4,7,10,13,16,19-hexaenamido)ethyl)-2-hydroxybenzamide

Designations withdrawn: EMEA/OD/096/05 2'-O-methyl-phosphorothioate oligonucleotide, EMEA/OD/025/06 2-(4-(diethylamino) phenyl)-6-methyl-2H-benzo[d][1,2,3] triazol-5-amine, EMA/OD/085/10 Recombinant fusion protein consisting of the extracellular portion of human activin receptor IIB linked to the human IgG1 Fc domain

2.2.10. - EMA/OD/102/16

Treatment of retinitis pigmentosa

Action: For adoption

Documents tabled:
Draft Summary report

Notes:
There have been 15 designations for this condition: Please see 2.2.1.

2.2.11. - EMA/OD/202/15

Treatment of variegate porphyria

Action: For adoption

Documents tabled:
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2.2.12. - EMA/OD/099/16

Treatment of periventricular leukomalacia

Action: For adoption

Documents tabled:
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2.2.13. - EMA/OD/087/16

Treatment of diffuse large B cell lymphoma

Action: For adoption

Documents tabled:
Draft Summary report

Notes:
There have been 10 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, EMEA/OD/091/08 Recombinant hisitidine-tagged idiotype immunoglobulin Fab fragment of clonal B-cell receptors, EMA/OD/160/10 Lenalidomide, EMA/OD/116/13 Ibrutinib, EMA/OD/092/14 obinutuzumab , EMA/OD/215/14 Humanised Fc engineered monoclonal antibody against CD19 , EMA/OD/005/15 Humanised anti-CD37 monoclonal antibody conjugated to maytansinoid DM1 , EMA/OD/084/15 2'-deoxyguanosylyl-(3',5'-phosphoryl)-2'-deoxythymidylyl-(3',5'-phosphoryl)- 2'-deoxyguanosylyl-(3',5'-phosphoryl)-2'-deoxycytidylyl-(3',5'-phosphoryl)-2'-deoxycytidylyl -(3',5'-phosphoryl)-2'-deoxycytidylyl-(3',5'-phosphoryl)-2'-deoxyguanosylyl-(3',5'-phosphoryl)-2'-deoxycytidilyl-(3',5'-phosphoryl)-2'-deoxycytidylyl-(3',5'-phosphoryl)-2'-deoxycytidylyl-(3',5'-phosphoryl)-2'-deoxycytidylyl-(3',5'-phosphoryl)-2'-deoxythymidylyl-(3',5'-phosphoryl)-2'-deoxyadenosyl-(3',5'-phosphoryl)-2'-deoxycytidylyl-(3',5'-phosphoryl)-2'-deoxyguanosylyl-(3',5'-phosphoryl)-2'-deoxycytidylyl-(3',5'-phosphoryl)-2'-deoxyguanosylyl-(3',5'-phosphoryl)-2'-deoxycytidylyl-(3',5'-phosphoryl)-2'-deoxyadenosyl-(3',5'-phosphoryl)-2'-deoxycytidylyl-(3',5'-phosphoryl)-2'-deoxyguanosylyl-2'-deoxycytidylyl-(3',5'-phosphoryl)-2'-deoxyadenosyl-(3',5'-phosphoryl)-2'-deoxycytidine, sodium salt, EMEA/OD/040/01 Thalidomide

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

2.2.14. - EMA/OD/110/16

Treatment of graft versus host disease

Action: For adoption

Documents tabled:
Draft Summary report

Notes:
There have been 10 designations for this condition: Please see 2.1.6.

2.2.15. - EMA/OD/101/16

Treatment of malignant mesothelioma

Action: For adoption

Documents tabled:
Draft Summary report

Notes:
There have been 9 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-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

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

2.2.16. - EMA/OD/105/16

Treatment of progressive multifocal leukoencephalopathy

Action: For adoption

Documents tabled:
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Notes:
There has been 1 designation for this condition: EMA/OD/011/12 Recombinant human interleukin-7

2.2.17. - EMA/OD/111/16

Treatment of cholangiocarcinoma

Action: For adoption

Documents tabled:
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Notes:
Designation withdrawn: EMEA/OD/024/04 Porfimer sodium (for use with photodynamic therapy)

2.2.18. - EMA/OD/112/16

Treatment of narcolepsy

Action: For adoption

Documents tabled:
Draft Summary report

Notes:
There have been 3 designations for this condition: EMEA/OD/087/06 1-{3-[3-(4-chlorophenyl)propoxy]propyl}piperidine, hydrochloride, EMA/OD/254/14 Mazindol, EMA/OD/002/15 Mazindol

Designation withdrawn: EMEA/OD/051/02 Sodium oxybate

2.2.19. - EMA/OD/092/16

Treatment of haemophilia B

Action: For adoption

Documents tabled:
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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)

2.2.20. - EMA/OD/093/16

Treatment of haemophilia A

Action: For adoption

Documents tabled:
Draft Summary report

Notes:
There have been 9 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

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)

2.2.21. - EMA/OD/094/16

Prevention of graft loss in pancreatic islet transplantation

Action: For adoption

Documents tabled:
Draft Summary report

2.2.22. - EMA/OD/089/16

Treatment of gastroenteropancreatic neuroendocrine tumours

Action: For adoption

Documents tabled:
Draft Summary report

Notes:
There have been 4 designations for this condition: EMA/OD/173/13 68Ga-2,2'-(7-(4-((S)-1-((4S,7S,10S,13R,16S,19R)-4-((R)-1-amino-3-(4-hydroxyphenyl)-1-oxopropan-2-ylcarbamoyl)-10-(4-aminobutyl)-16-(4-((S)-2,6-dioxohexahydropyrimidine-4-carboxamido)benzyl)-7-((R)-1-hydroxyethyl)-6,9,12,15,18-pentaoxo-13-(4-ureidobenzyl)-

1,2-dithia-5,8,11,14,17-pentaazacycloicosan-19-ylamino)-3-(4-chlorophenyl)-1-oxopropan-2-ylamino)-1-carboxy-4-oxobutyl)-1,4,7-triazonane-1,4-diyl)diacetic acid, EMA/OD/219/14 Gallium (68Ga)-edotreotide, EMEA/OD/005/04 (2-aminoethyl) carbamic acid (2R,5S,8S,11S,14R,17S,19aS)-11-(4-aminobutyl)-5-benzyl-8-(4-benzyloxy benzyl)-14-(1H-indol-3-ylmethyl)-4,7,10,13,16,19-hexaoxo-17-phenyloctadecahydro-3a,6,9,12,15,18-hexaazacyclopentacyclooctadecen-2-yl ester, di[(S)-2-aminosuccinic acid] salt, EMA/OD/211/15 Fosbretabulin tromethamine

2.2.23. - EMA/OD/086/16

Treatment of alkaptonuria

Action: For adoption

Documents tabled:
Draft Summary report

Notes:
There has been 1 designation for this condition: EMEA/OD/071/01 Nitisinone

2.2.24. - EMA/OD/091/16

Treatment of cutaneous T-cell lymphoma

Action: For adoption

Documents tabled:
Draft Summary report

Notes:
There have been 13 designations for this condition: EMEA/OD/038/01 Denileukin diftitox, EMEA/OD/001/04 Human monoclonal antibody against CD4 , EMEA/OD/001/05 (E)-(1S,4S,10S,21R)-7-[(Z)-ethylidene]-4,21-diisopropyl-2-oxa-12,13-dithia-5,8,20,23-tetraazabicyclo[8.7.6]tricos-16-ene-3,6,9,19,22-pentone, EMEA/OD/030/08 Miltefosine, EMEA/OD/135/09 Pralatrexate, EMA/OD/112/11 Chlormethine, EMA/OD/100/11 Brentuximab vedotin, EMA/OD/050/12 Naloxone hydrochloride dihydrate , EMA/OD/066/12 Recombinant anti-CD3-bi-single-chain-Fv-diphtheria toxin fusion protein , EMA/OD/084/14 Humanised IgG1 monoclonal antibody against human KIR3DL2 , EMA/OD/033/15 Synthetic hypericin , EMA/OD/254/15 Resiquimod, EMA/OD/203/15 Fenretinide

Designations withdrawn: EMEA/OD/007/03 Adenovirus-Interferon gamma-coding DNA sequence, EMEA/OD/003/04 Suberolylanilide Hydroxamic acid, EMEA/OD/015/07 Panobinostat lactate

2.2.25. - EMA/OD/115/16

Treatment of myelofibrosis

Action: For adoption

Documents tabled:
Draft Summary report

Notes:

There has been 1 designation for this condition: EMA/OD/154/15 Imetelstat sodium

2.2.26. - EMA/OD/126/16

Treatment of Duchenne muscular dystrophy

Action: For adoption

Documents tabled:
Draft Summary report

Notes:
There have been 24 designations for this condition: EMEA/OD/106/04 3-[5-(2-fluoro-phenyl)-[1,2,4]oxadiazole-3-yl]-benzoic acid, EMEA/OD/026/05 Adeno-associated viral vector containing a modified U7 snRNA gene, EMEA/OD/077/06 Idebenone, EMEA/OD/065/08 5-(ethylsulfonyl)-2-(naphthalen-2-yl)benzo[d]oxazole, EMEA/OD/049/08 RNA, [P-deoxy-P-(dimethylamino)] (2',3'-dideoxy-2',3'-imino-2',3'-seco) (2'a→5') (C-m5U-C-C-A-A-C-A-m5U-C-A-A-G-G-A-A-G-A-m5U-G-G-C-A-m5U-m5U-m5U-C-m5U-A-G), P-[4-[[2-[2-(2-hydroxyethoxy)ethoxy]ethoxy]carbonyl]-1-piperazinyl] N,N-dimethylam, EMEA/OD/081/08 Exon 44 specific phosphorothioate oligonucleotide, EMEA/OD/082/08 Exon 51 specific phosphorothioate oligonucleotide, EMEA/OD/044/09 Adeno-associated viral vector containing modified U1 snRNA, EMEA/OD/083/09 RNA, [P-deoxy-P-(dimethylamino)] (2',3'-dideoxy-2',3'-imino-2',3'-seco) (2'a→5') (C-m5U-m5U-A-C-A-G-G-C-m5U-C-C-A-A-m5U-A-G-m5U-G-G-m5U-C-A-G-m5U), 5' [P-[4-[[2-[2-(2-hydroxyethoxy)ethoxy]ethoxy]carbonyl]-1-piperazinyl]-N,N-dimethylaminophosphonamidate], 3'-[2'a-[N2-acetyl-L-arginyl-6-aminohexanoyl-L-arginyl-L-arginyl-β-alanyl-L-arginyl-L-arginyl-6-aminohexanoyl-L-arginyl-L-arginyl-β-alanyl-L-arginyl-6-aminohexanoyl-β-alanyl], octahydrochloride, EMA/OD/090/13 Naproxcinod, EMA/OD/142/11 Exon 45 specific phosphorothioate oligonucleotide, EMA/OD/143/11 Exon 53 specific phosphorothioate oligonucleotide , EMA/OD/162/11 Halofuginone hydrobromide , EMA/OD/028/12 Givinostat, EMA/OD/121/12 Exon 52 specific phosphorothioate oligonucleotide , EMA/OD/122/12 Exon 55 specific phosphorothioate oligonucleotide , EMA/OD/164/12 Humanised monoclonal antibody against myostatin , EMA/OD/183/12 R,S-O-(3-piperidino-2-hydroxy-1-propyl)-nicotinic acid amidoxime dihydrochloride , EMA/OD/162/13 Asp-Arg-Val-Tyr-Ile-His-Pro , EMA/OD/049/14 17α,21-dihydroxy-16α-methyl-pregna-1,4,9(11)-triene-3,20-dione , EMA/OD/166/14 Adeno-associated viral vector serotype 8 containing the human MD1 gene , EMA/OD/307/14 Rimeporide, EMA/OD/041/15 Allogeneic human adult stem cells, isolated from skeletal muscle and expanded ex vivo , EMA/OD/109/15 N-(2-((4Z,7Z,10Z,13Z,16Z,19Z)-docosa-4,7,10,13,16,19-hexaenamido)ethyl)-2-hydroxybenzamide

Designations withdrawn: EMEA/OD/096/05 2'-O-methyl-phosphorothioate oligonucleotide, EMEA/OD/025/06 2-(4-(diethylamino) phenyl)-6-methyl-2H-benzo[d][1,2,3] triazol-5-amine, EMA/OD/085/10 Recombinant fusion protein consisting of the extracellular portion of human activin receptor IIB linked to the human IgG1 Fc domain

2.2.27. - EMA/OD/095/16

Treatment of systemic sclerosis

Action: For adoption

Documents tabled:
Draft Summary report

Notes:
There have been 14 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

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

2.2.28. - EMA/OD/090/16

Treatment of acute myeloid leukaemia

Action: For adoption

Documents tabled:
Draft Summary report

Notes:
There have been 48 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/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/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-Ph….. , 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

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 Ig-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/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

2.2.29. - EMA/OD/098/16

Treatment of glycogen storage disease type 2 (Pompe disease)

Action: For adoption

Documents tabled:
Draft Summary report

Notes:
There have been 3 designations for this condition: EMA/OD/148/13 recombinant human alpha-glucosidase conjugated with multiple copies of synthetic bismannose-6-phosphate-tetra-mannose glycan, EMA/OD/057/11 Glycosylation independent lysosomal targeting tagged recombinant human acid alpha glucosidase', EMA/OD/018/12 Recombinant adeno-associated viral vector containing human acid alfa-glucosidase-gene

Designations withdrawn: EMEA/OD/001/07 Recombinant adeno-associated viral vector containing human acid alfa-glucosidase-gene

2.2.30. - EMA/OD/116/16

Treatment of acute radiation syndrome

Action: For adoption

Documents tabled:
Draft Summary report

Notes:
There have been 2 designations for this condition: EMA/OD/060/15 Fibrinogen-coated albumin spheres, EMA/OD/191/15 Entolimod

2.2.31. - EMA/OD/118/16

Treatment of graft-versus-host disease

Action: For adoption

Documents tabled:
Draft Summary report

Notes:
There have been 10 designations for this condition: Please see 2.1.6.

2.2.32. - EMA/OD/107/16

Treatment of paroxysmal nocturnal haemoglobinuria

Action: For adoption

Documents tabled:
Draft Summary report

Notes:
There have been 3 designations for this condition: EMEA/OD/042/03 Eculizumab, EMA/OD/098/14 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) , EMA/OD/246/15 Fc- and CDR-modified humanised monoclonal antibody against C5

Designation withdrawn: EMEA/OD/016/02 Myristolated-peptidyl-recombinant Human CD59

2.2.33. - EMA/OD/125/16

Treatment of acute hepatic porphyria

Action: For adoption

Documents tabled:
Draft Summary report

2.2.34. - EMA/OD/084/16

Treatment of short bowel syndrome

Action: For adoption

Documents tabled:
Draft Summary report

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)

2.2.35. - EMA/OD/120/16

Treatment of amyotrophic lateral sclerosis

Action: For adoption

Documents tabled:
Draft Summary report

Notes:
There have been 16 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

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

2.2.36. - EMA/OD/085/16

Treatment of glioma

Action: For adoption

Documents tabled:
Draft Summary report

Notes:
There have been 41 designations for this condition: Please see 2.1.14.

2.2.37. - EMA/OD/121/16

Treatment of multiple myeloma

Action: For adoption

Documents tabled:
Draft Summary report

Notes:
There have been 13 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

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

2.2.38. - EMA/OD/122/16

Treatment of diffuse large B-cell lymphoma

Action: For adoption

Documents tabled:
Draft Summary report

Notes:

There have been 10 designations for this condition: Please see 2.2.13.

2.2.39. - EMA/OD/109/16

Treatment of global ischaemic reperfusion Injury

Action: For adoption

Documents tabled:
Draft Summary report

2.3. Revision of the COMP opinions

2.3.1. Melatonin - EMA/OD/001/16

Therapicon Srl; Treatment of necrotising enterocolitis

COMP coordinator: Vallo Tillmann

Action: For discussion

Document tabled:
Letter from EC dated 01-07-2016

2.4. COMP opinions adopted via written procedure following previous meeting

None

2.5. Appeal

None

2.6. Nominations

2.6.1. New applications for orphan medicinal product designation - Appointment of COMP coordinators

Action: For adoption

Document tabled:
OMPD applications - appointment of coord. at the 11-13 July 2016 COMP meeting

2.7. Evaluation on-going

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

Action: For information

Notes:
Cross reference to other agenda point. See 6.8.1. Table 6. Evaluation Ongoing.

3. Requests for protocol assistance with significant benefit question

3.1. Ongoing procedures

3.1.1. -

Treatment of microscopic polyangiitis

Action: For discussion

3.1.2. -

Treatment of granulomatosis with polyangiitis

Action: For discussion

3.1.3. -

Treatment of soft tissue sarcoma

Action: For discussion

3.2. Finalised letters

3.2.1. -

Treatment of acute myeloid leukaemia

Action: For information

3.2.2. -

Treatment of growth hormone deficiency

Action: For information

3.3. New requests

3.3.1. -

Treatment of tuberculosis

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. Zalmoxis - allogeneic T cells genetically modified to express suicide gene - EMEA/OD/041/03, EU/3/03/168, EMEA/H/C/002801

MolMed SpA; Adjunctive treatment in haematopoietic cell transplantation

COMP coordinator: Armando Magrelli and Violeta Stoyanova

Action: For information

Documents tabled:
Report on review of OMPD

Notes:
Status of the procedure at the CAT/CHMP: Opinion adopted in June 2016
COMP positive opinion was adopted by Written Procedure after its June meeting.

4.1.2. Revlimid – lenalidomide - Type II variation - EMA/OD/078/11, EU/3/11/924, EMEA/H/C/000717/II/0079

Celgene Europe Limited; Treatment of mantle cell lymphoma

COMP coordinator: Jens Ersbøll and Daniel O'Connor; Patient's expert: Guy Bouguet; CHMP rapporteur: Pierre Demolis; CHMP co-rapporteur: Filip Josephson

Action: For information

Documents tabled:
Revised draft Summary report

Notes:
Status of the procedure at the CHMP: Opinion adopted in January 2016.
Appeal of the COMP negative opinion adopted in February 2016.
COMP final positive opinion was adopted by Written Procedure after its June meeting.

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

4.2.1. - chenodeoxycholic acid – EMA/OD/196/14, EU/3/14/1406, EMEA/H/C/004061

Sigma-tau Arzneimittel GmbH; Treatment of inborn errors of primary bile acid synthesis

Action: For information, Oral explanation delayed

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

4.2.2. - parathyroid hormone – EMA/OD/102/13, EU/3/13/1210, EMEA/H/C/003861

NPS Pharma Holdings Limited; Treatment of hypoparathyroidism

Action: For discussion

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

4.2.3. – olaratumab – EMA/OD/266/14, EU/3/15/1447, EMEA/H/C/004216

Eli Lilly Nederland B.V.; Treatment of soft tissue sarcoma

Action: For discussion

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

4.2.4. - irinotecan - EMA/OD/051/11, EU/3/11/933, EMEA/H/C/004125

Baxter Innovations GmbH; Treatment of pancreatic cancer

Action: For discussion

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

4.3. On-going procedures

Action: For information

4.4. Public Summary of Opinion

Action: For information

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

None

6. Organisational, regulatory and methodological matters

6.1. Mandate and organisation of the COMP

6.1.1. Strategic Review & Learning meetings

Strategic Review & Learning meeting in Utrecht (NL), 31 May-1 June 2016

Action: For information

Document tabled:
Minutes of CHMP COMP strategic and learning meeting NL 2016

6.1.2. Protocol Assistance Working Group

Proposed meeting time on 12 July 2016 at time 18:00, room 2H

6.1.3. COMP Drafting Group

Proposed meeting time on 11 July 2016 at time 18:00, room 2H

6.2. Coordination with EMA Scientific Committees or CMDh-v

6.2.1. PDCO/COMP Working Group

Proposed meeting time on 13 July 2016 at time 13:00, room 10A

6.2.2. Recommendations on eligibility to PRIME – report from CHMP

Action: For information

Document tabled:
PRIME eligibility requests - list of adopted outcomes June 2016

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

6.3.1. Scientific Advice Working Party (SAWP)

Revised SAWP Mandate

Action: For information

Document tabled:
Mandate of the Scientific Advice Working Party EMEA/CHMP/SAWP/6968604 rev 10

6.4. Cooperation within the EU regulatory network

6.4.1. Commission Expert Group on Rare Diseases

Report from the Commission Expert Group on Rare Diseases 7th meeting held on 5-6 April 2016

Action: For information

6.5. Cooperation with International Regulators

6.5.1. Food and Drug Administration (FDA)

Action: For information

Document tabled:
Draft Agenda June 21 2016

Notes:
Monthly teleconference

6.5.2. Japanese Pharmaceuticals and Medical Devices Agency (PMDA)

None

6.5.3. The Therapeutic Goods Administration (TGA), Australia

None

6.5.4. Health Canada

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 tabled:
COMP Work Plan 2016
6-7-1 COMP Work plan tracking tool 2016

6.7.2. COMP Work Plan 2017

Action: For information

Document 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. Request for COMP opinion on therapeutic indication proposed for upcoming MAA

Action: For discussion

Document tabled:
7-1 EMA COMP opinion request - June 2016

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
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/