21 May 2018
EMA/COMP/251740/2018
Inspections, Human Medicines Pharmacovigilance and Committees

Committee for Orphan Medicinal Products (COMP)

Draft agenda for the meeting on 22-24 May 2018

Chair: Bruno Sepodes – Vice-Chair: Lesley Greene

22 May 2018, 09:00-19:30, room 2F

23 May 2018, 08:30-19:30, room 2F

24 May 2018, 08:30-13:00, 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).

30 Churchill Place ● Canary Wharf ● London E14 5EU ● United Kingdom
Telephone +44 (0)20 3660 6000 **Facsimile** +44 (0)20 3660 5555
Send a question via our website www.ema.europa.eu/contact

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1. Introduction

1.1. Welcome and declarations of interest of members and experts

Pre-meeting list of participants and restrictions in relation to declarations of interests applicable to the items of the agenda for the COMP plenary session to be held 22-24 May 2018. See May 2018 COMP minutes (to be published post June 2018 COMP meeting).

1.2. Adoption of agenda

COMP agenda for 22-24 May 2018.

1.3. Adoption of the minutes

COMP minutes for 17-19 April 2018.

2. Applications for orphan medicinal product designation

2.1. For opinion

2.1.1. - EMA/OD/001/18

Treatment of transthyretin-mediated amyloidosis

Action: For information

Document(s) tabled:
Withdrawal request of 27 April 2018

Notes: There have been 3 designations for this condition: EMA/OD/142/10 Synthetic double-stranded siRNA oligonucleotide directed against transthyretin mRNA, EMA/OD/194/13 Synthetic double-stranded siRNA oligonucleotide directed against transthyretin mRNA and covalently linked to a ligand containing three N-acetylgalactosamine residues, EMA/OD/098/13 Phosphorothioate oligonucleotide targeted to transthyretin

2.1.2. - EMA/OD/002/18

Treatment of transthyretin-mediated amyloidosis

Action: For information

Document(s) tabled:
Withdrawal request of 27 April 2018

Notes: There have been 3 designations for this condition: See 2.1.1

2.1.3. - EMA/OD/014/18

Treatment of epidermolysis bullosa

Action: For adoption, Oral explanation to be held on 22 May 2018 at 14:30

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

Notes: There have been 15 designations for this condition: EMEA/OD/111/05 Bilayer engineered skin composed of keratinocytes from the patient (autologous) and fibroblasts from a donor (allogeneic) embedded in a plasma matrix, EMEA/OD/061/09 Allogeneic human dermal fibroblasts, EMA/OD/120/10 Dry extract from birch bark (DER 0.1-0.2:1), extraction solvent n-heptane 95% (V/V), EMA/OD/145/13 Allantoin, EMA/OD/149/13 Diacerein, EMA/OD/201/13 Recombinant human alpha 1 chain homotrimer of type VII collagen, EMA/OD/197/14 Allogeneic adipose-derived adult mesenchymal stem cells contained in a fibrin-based bioengineered dermis, EMA/OD/218/15 Autologous dermal fibroblasts genetically modified ex vivo with a lentiviral vector containing the human COL7A1 gene, EMA/OD/299/14 Ex-vivo-expanded autologous human keratinocytes containing epidermal stem cells transduced with a COL17A1-encoding retroviral vector, EMA/OD/297/14 Ex-vivo-expanded autologous human keratinocytes containing epidermal stem cells transduced with a LAMB3-encoding retroviral vector, EMA/OD/188/15 Ex-vivo-expanded autologous fibroblasts transduced with lentiviral vector containing the COL7A1 gene, EMA/OD/283/16 Ex-vivo-expanded autologous keratinocytes transduced with retroviral vector containing the COL7A1 gene, EMA/OD/031/17 Asp-Arg-Val-Tyr-Ile-His-Pro, EMA/OD/140/17 Antisense oligonucleotide targeting exon 73 in the COL7A1 gene, EMA/OD/244/17 Genetically modified replication- incompetent herpes simplex virus-1 expressesing collagen VII
Designation withdrawn: EMA/OD/172/10 Human dermal fibroblasts cultured on a bioresorbable polyglactin mesh

2.1.4. - EMA/OD/015/18

Treatment of malignant cerebral oedema

Action: For information

Document(s) tabled:
Withdrawal request of 4 May 2018

2.1.5. - EMA/OD/003/18

Treatment of hereditary angioedema

Action: For adoption, Oral explanation to be held on 22 May 2018 at 17:00

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

Notes: There has been 1 designation for this condition: EMA/OD/075/15 Recombinant human IgG1 kappa light chain monoclonal antibody targeting plasma kallikrein
Designation withdrawn: EMA/OD/170/14 3-[2-(4-carbamimidoyl-phenylcarbamoyl)-5-methoxy-4-vinyl-phenyl]-6-(cyclopropylmethyl-carbamoyl)-pyridine-2-carboxylic acid

2.1.6. - EMA/OD/009/18

Treatment of growth hormone deficiency

Action: For adoption, Oral explanation to be held on 23 May 2018 at 09:30

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

Notes: There have been 3 designations for this condition: EMA/OD/133/12 Recombinant modified human growth hormone, EMA/OD/074/13 Recombinant human growth hormone modified by fusion with two hydrophilic polypeptide chains, EMA/OD/013/17 Ibutamoren mesilate

2.1.7. - EMA/OD/012/18

Treatment of cystinuria

Action: For adoption, Oral explanation to be held on 23 May 2018 at 11:00

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

2.1.8. - EMA/OD/007/18

Treatment of haematopoietic stem cell transplantation

Action: For adoption, Oral explanation to be held on 23 May 2018 at 12:00

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

Notes: There have been 8 designations for this condition: EMA/OD/150/17 Allogeneic CD4+ and CD25+ T lymphocytes ex vivo incubated with GP120, EMA/OD/149/16 Allogeneic peripheral blood mononuclear cells incubated ex vivo with 16, 16-dimethyl prostaglandin E2 and dexamethasone, EMA/OD/191/16 Human donor haematopoietic stem and progenitor cells that have been treated ex vivo with the protein transduction domain of the HIV-1 transactivation protein fused to MYC transcription factor, EMA/OD/257/16 Allogeneic, ex vivo expanded, umbilical cord blood-derived, hematopoietic CD34+ progenitor cells and allogeneic, non-expanded, umbilical cord blood-derived, hematopoietic mature myeloid and lymphoid cells, EMA/OD/090/16 Radio-iodinated (131I) anti-CD45 murine monoclonal antibody, EMA/OD/020/16 Allogeneic donor-derived ex-vivo expanded T lymphocytes transduced with a retroviral vector containing inducible caspase 9 and truncated CD19, EMA/OD/008/16 Donor T lymphocytes depleted ex vivo of host alloreactive T cells using photodynamic treatment, EMA/OD/192/17 Allogeneic umbilical cord blood CD34+ cells cultured ex vivo with Notch ligand Delta1

2.1.9. - EMA/OD/005/18

Treatment of progressive supranuclear palsy

Action: For adoption, Oral explanation to be held on 23 May 2018 at 14:30

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

Notes: There have been 5 designations for this condition: EMA/OD/076/10 Methylthioninium, EMA/OD/261/14 N-(3-(4-(3-(diisobutylamino)propyl)piperazin-1-

yl)propyl)-1H-benzo[d]imidazol-2-amine disulphate salt, EMA/OD/044/15 Humanised IgG4 monoclonal antibody against extracellular tau, EMA/OD/193/15 Tolfenamic acid, EMA/OD/239/15 Humanised recombinant IgG4 anti-human tau antibody
Designations withdrawn: EMEA/OD/074/09 4-benzyl-2-naphtalen-1-yl-1,2,4-thiadiazolidine-3,5-dione, EMEA/OD/129/09 Davunetide

2.1.10. - EMA/OD/020/18

Treatment of Duchenne muscular dystrophy

Action: For adoption, Oral explanation to be held on 23 May 2018 at 15:30

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

Notes: There have been 27 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-ydroxyethoxy)ethoxy]ethoxy]carbonyl]-1-piperazinyl] N,N dimethylaminophosphonamidate, 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/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, EMA/OD/161/16 Recombinant adeno-associated viral vector encoding a human micro-dystrophin gene under the control of a muscle specific promoter, EMA/OD/096/16 Adeno-associated viral vector serotype 9 containing the human mini-dystrophin gene, EMA/OD/133/17 Tamoxifen citrate, EMA/OD/154/17 Metformin and L-citrulline
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, EMA/OD/090/13 Naproxcinod

2.1.11. - EMA/OD/006/18

Treatment of neurodegeneration with brain iron accumulation

Action: For adoption, Oral explanation to be held on 23 May 2018 at 17:00

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

2.2. For discussion / preparation for an opinion

2.2.1. - EMA/OD/004/18

Treatment of tuberculosis

Action: For discussion/adoption

Document(s) tabled:
Draft Summary report

Notes: There have been 9 designations for this condition: EMEA/OD/024/05 (1R,2S) 6-bromo-alpha-[2-(dimethylamino)ethyl]-2-methoxy-alpha-(1-naphthyl)-beta-phenyl-3-quinolineethanol, EMEA/OD/036/07 N-adamantanyl-N'-Geranyl-ethylenediamine, EMEA/OD/074/07 (S)-2-nitro-6-(4-trifluoromethoxy)benzyloxy)-6,7-dihydro-5H-imidazo[2,1-b] [1,3] oxazine, EMEA/OD/094/07 (R)-2-Methyl-6-nitro-2-{4-[4-(4-trifluoromethoxyphenoxy)piperidin-1-yl]phenoxymethyl}-2,3-dihydroimidazo[2,1-b]oxazole, EMEA/OD/145/09 Rifapentine, EMA/OD/033/10 Heat-killed Mycobacterium vaccae (whole cell), EMA/OD/072/10 Para-aminosalicylic acid, EMA/OD/029/11 N-{[(5S)-3-(3-fluoro-4-thiomorpholin-4-ylphenyl)-2-oxo-1,3-oxazolidin-5-yl]methyl}acetamide, EMA/OD/098/17 Purified pasteurised and freeze-dried cell-wall fragments from Mycobacterium tuberculosis strain RUTI

2.2.2. - EMA/OD/030/18

Treatment of idiopathic pulmonary fibrosis

Action: For discussion/adoption

Document(s) tabled:
Draft Summary report

Notes: There have been 11 designations for this condition: EMEA/OD/052/04 Pirfenidone, EMEA/OD/054/07 Interferon gamma, EMEA/OD/104/09 Macitentan, EMA/OD/079/10 2-(2-chlorophenyl)-4-[3-(dimethylamino)phenyl]-5-methyl-1H-pyrazolo[4,3-C]pyridine-3,6(2H,5H)-dione, EMA/OD/048/12 Recombinant human pentraxin-2, EMA/OD/186/12 nintedanib, EMA/OD/051/14 Humanised anti-alpha v beta 6 monoclonal antibody, EMA/OD/130/14 1-(6-benzothiazolylsulfonyl)-5-chloro-1H-indole-2-butanoic acid, EMA/OD/072/15 3-pentylbenzeneacetic acid sodium salt, EMA/OD/046/16 3-[4-(1H-imidazol-1-ylmethyl)phenyl]-5-(2-methylpropyl)thiophene-2-[(N-butyloxylcarbamate)-sulphonamide] sodium salt, EMA/OD/088/16 2-((2-ethyl-6-(4-(2-(3-hydroxyazetidin-1-yl)-

2-oxoethyl)-piperazin-1-yl)-8-methylimidazo[1,2-alpha]pyridin-3-yl)-(methyl)amino)-4-(4-fluorophenyl)-thiazole-5-carbonitrile

Designations withdrawn: EMEA/OD/002/05 Interferon gamma, EMEA/OD/033/04 Heparin-Sodium, EMEA/OD/075/04 Acetylcysteine, EMEA/OD/105/07 Recombinant human monoclonal antibody against transforming growth factor beta-1, 2 and 3, EMEA/OD/027/08 Bosentan, EMA/OD/029/10 Ambrisentan, EMA/OD/091/11 4-[[9-[(3S)-tetrahydro-3-furanyl]-8-[(2,4,6-trifluorophenyl)amino]-9H-purin-2-yl]amino]-trans-cyclohexanol, EMA/OD/111/12 Tralokinumab

2.2.3. - EMA/OD/031/18

Treatment of perinatal asphyxia

Action: For discussion/adoption

Document(s) tabled:
Draft Summary report

Notes: There have been 7 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, EMA/OD/220/16 Pr-D-Cys-Met-Pip-Arg-Leu-Arg-Sar-Cys-Lys-Arg-Pro-Tyr-Tle-Leu-OH

2.2.4. - EMA/OD/022/18

Treatment of ornithine transcarbamylase deficiency

Action: For discussion/adoption

Document(s) tabled:
Draft Summary report

Notes: There have been 6 designations for this condition: EMEA/OD/101/07 Heterologous human adult liver derived stem cells, EMA/OD/026/11 Heterologous human adult liver-derived stem cells, EMA/OD/227/15 Adeno-associated viral vector serotype 8 encoding human ornithine transcarbamylase, EMA/OD/053/16 Sodium benzoate, EMA/OD/310/16 Adeno-associated viral vector serotype LK03 encoding human ornithine transcarbamylase, EMA/OD/326/16 Modified messenger ribonucleic acid encoding human ornithine transcarbamylase enzyme encapsulated into lipid nanoparticles
Designation withdrawn: EMA/OD/097/11 Sodium phenybutyrate

2.2.5. - EMA/OD/036/18

Treatment of glioma

Action: For discussion/adoption

Document(s) tabled:
Draft Summary report

Notes: There have been 43 designations for this condition: EMEA/OD/026/03 Herpes simplex virus lacking infected cell protein 34.5, EMEA/OD/055/03 Gimatecan, EMEA/OD/050/04 Biotinylated anti-tenascin monoclonal antibody for use with 90-Yttrium,

EMEA/OD/038/04 Anti epidermal growth factor receptor antibody h-R3, EMEA/OD/030/05 Oligonucleotide phosphorothioate (TAAACGTTATAACGTTATGACGTCAT), sodium salt, EMEA/OD/068/05 Enzastaurin hydrochloride, EMEA/OD/110/05 4-[131I] iodo-L-phenylalanine, EMEA/OD/081/06 Autologous dendritic cells pulsed with autologous tumour cell lysate, EMEA/OD/038/07 Iodine (131I) Chlorotoxin, EMEA/OD/004/08 Recombinant fusion protein of circulary-permuted IL-4 and pseudomonas exotoxin A, [IL-4(38-37)-PE38KDEL], EMEA/OD/023/08 Topotecan hydrochloride (liposomal), EMEA/OD/034/08 Gadodiamide (liposomal), EMEA/OD/104/08 Autologous tumour-derived gp96 heat shock protein-peptide complex, EMEA/OD/098/09 Recombinant fusion protein consisting of the extracellular portion of CD95 fused to the Fc part of a human IgG1 molecule, EMA/OD/086/10 7-beta-hydroxycholesteryl-3-beta-oleate, EMA/OD/092/12 IL-12-secreting dendritic cells, loaded with autologous tumour lysate, EMA/OD/077/11 L-cysteine, L-leucyl-L-alpha-glutamyl-L-alpha-glutamyl-L-lysyl-L-lysylglycyl-L-asparaginyl-L-tyrosyl-L-valyl-L-valyl-L-threonyl-L-alpha-aspartyl-L-histidyl-S-[1-[(4-carboxycyclohexyl)methyl]-2,5-dioxo-3-pyrrolidinyl]-complex with keyhole limpet haemocyanin, EMA/OD/050/11 2-hydroxyoleic acid, EMA/OD/157/11 Adenovirus-associated vector containing human Fas-c gene, EMA/OD/170/12 4-[2-(6-methylpyridin-2-yl)-5,6-dihydro-4H-pyrrolo[1,2-b]pyrazol-3-yl]-quinoline-6-carboxamide monohydrate, EMA/OD/148/12 1,2:5,6-Dianhydrogalactitol, EMA/OD/086/13 Autologous ex vivo expanded leukocytes treated with 5-aza-2'-deoxycytidine, EMA/OD/001/14 Autologous dendritic cells pulsed with RNA from glioma stem cells, EMA/OD/107/13 Allogeneic and autologous haptenised and irradiated cells and cell lysates derived from glioma, EMA/OD/174/13 Autologous dendritic cells pulsed with tumour antigen-derived synthetic peptides (MAGE-1, HER-2, AIM-2, TRP-2, gp-100, and interleukin-13 receptor alpha), EMA/OD/111/14 Recombinant human bone morphogenetic protein 4, EMA/OD/003/14 Paclitaxel-succinate-Arg-Arg-Leu-Ser-Tyr-Ser-Arg-Arg-Arg-Phe, EMA/OD/065/14 Humanised recombinant monoclonal antibody against epidermal growth factor receptor conjugated to maleimidocaproyl monomethylauristatin F, EMA/OD/132/14 Olaptesed pegol, EMA/OD/200/14 5,5'-(4-(trifluromethyl)benzylazanediyl)bis(methylene)diquinolin-8-ol, EMA/OD/159/14 Chloroquine, EMA/OD/176/14 Adenovirus serotype 5 containing partial E1A deletion and an integrin-binding domain, EMA/OD/251/14 Recombinant human glutamate oxaloacetate transaminase 1, EMA/OD/206/15 N-(4-Methoxyphenyl)-N,2,6-trimethylfuro[2,3-d]pyrimidin-4-amine, EMA/OD/009/16 Eflornithine, EMA/OD/222/15 Delta-9-tetrahydrocannabinol and cannabidiol from extracts of the Cannabis sativa L. plant, EMA/OD/067/16 Zoledronic acid, EMA/OD/085/16 Temozolomide, EMA/OD/068/17 Picropodophyllin, EMA/OD/215/16 5-aminolevulinic acid, EMA/OD/069/17 Salmonella typhi Ty21a strain transfected with a plasmid vector encoding the human vascular endothelial growth factor receptor 2, EMA/OD/185/17 Vocimagene amiretrorepvec, EMA/OD/198/17 Flucytosine

Designations withdrawn: EMEA/OD/004/02 Pseudomonas exotoxin (domains II/III)-Interleukin 13 chimeric protein, EMEA/OD/074/01 Human transferrin conjugated to mutant diptheria toxin, EMEA/OD/067/01 Carmustine (solution for intratumoral injection), EMEA/OD/050/06 Iodine (131I) anti-tenascin monoclonal antibody 81C6, EMEA/OD/037/02 Iodine (131I) anti-nucleohistone H1 chimeric biotinylated monoclonal antibody, EMEA/OD/067/03 Cilengitide, EMEA/OD/050/07 Doxorubicin hydrochloride (drug eluting beads), EMEA/OD/051/07 Irinotecan hydrochloride (drug eluting beads), EMEA/OD/112/08 Talampanel, EMEA/OD/004/09 4,6,8-trihydroxy-10-(3,7,11-trimethyldodeca-2,6,10-trienyl)-5,10-dihydrodibenzo[b,e][1,4] diazepin-11-one, EMA/OD/031/10 Glutathione-pegylated liposomal doxorubicin hydrochloride, EMA/OD/049/12 Humanised monoclonal antibody

against epidermal growth factor receptor, EMA/OD/019/12 Doxorubicin (administered after synthetic double-stranded siRNA oligonucleotide directed against claudin-5 complexed with polyethyleneimine), EMA/OD/136/12 Synthetic double-stranded siRNA oligonucleotide directed against Claudin-5 complexed with polyethyleneimine (prior to administration of doxorubicin), EMA/OD/113/15 Dronabinol and cannabidiol

2.2.6. - EMA/OD/028/18

Treatment of Duchenne muscular dystrophy

Action: For discussion/adoption

Document(s) tabled:
Draft Summary report

Notes: There have been 27 designations for this condition: See 2.1.10

2.2.7. - EMA/OD/108/17

Treatment of abdominal aortic aneurysm

Action: For discussion/adoption

Document(s) tabled:
Draft Summary report

2.2.8. - EMA/OD/024/18

Treatment in haematopoietic stem cell transplantation

Action: For discussion/adoption

Document(s) tabled:
Draft Summary report

Notes: There have been 8 designations for this condition: EMA/OD/008/16 Donor T lymphocytes depleted ex vivo of host alloreactive T cells using photodynamic treatment, EMA/OD/020/16 Allogeneic donor-derived ex-vivo expanded T lymphocytes transduced with a retroviral vector containing inducible caspase 9 and truncated CD19, EMA/OD/090/16 Radio-iodinated (131I) anti-CD45 murine monoclonal antibody, EMA/OD/149/16 Allogeneic peripheral blood mononuclear cells incubated ex vivo with 16, 16-dimethyl prostaglandin E2 and dexamethasone, EMA/OD/191/16 Human donor haematopoietic stem and progenitor cells that have been treated ex vivo with the protein transduction domain of the HIV-1 transactivation protein fused to MYC transcription factor, EMA/OD/257/16 Allogeneic, ex vivo expanded, umbilical cord blood-derived, hematopoietic CD34+ progenitor cells and allogeneic, non-expanded, umbilical cord blood-derived, hematopoietic mature myeloid and lymphoid cells, EMA/OD/150/17 Allogeneic CD4+ and CD25+ T lymphocytes ex vivo incubated with GP120, EMA/OD/192/17 Allogeneic umbilical cord blood CD34+ cells cultured ex vivo with Notch ligand Delta1

2.2.9. - EMA/OD/253/17

Treatment of pilonidal sinus disease

Action: For discussion/adoption

Document(s) tabled:
Draft Summary report

2.2.10. - EMA/OD/033/18

Treatment of Friedreich's ataxia

Action: For discussion/adoption

Document(s) tabled:
Draft Summary report

Notes: There have been 5 designations for this condition: EMEA/OD/037/01 Idebenone, EMEA/OD/082/03 Idebenone, EMA/OD/026/10 N-(6-(2-aminophenylamino)-6-oxohexyl)-4-methylbenzamide, EMA/OD/084/11 Interferon gamma, EMA/OD/219/17 Dimethyl fumarate

2.2.11. - EMA/OD/025/18

Treatment of Multiple Osteochondromas

Action: For discussion/adoption

Document(s) tabled:
Draft Summary report

2.2.12. - EMA/OD/027/18

Treatment of Mucopolysaccharidosis Type 1

Action: For discussion/adoption

Document(s) tabled:
Draft Summary report

Notes: There have been 4 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, EMA/OD/119/16 6'-(R)-methyl-5-O-(5-amino-5,6-dideoxy-α-L-talofuranosyl)-paromamine sulfate

2.2.13. - EMA/OD/035/18

Treatment of Pre-eclampsia

Action: For discussion/adoption

Document(s) tabled:
Draft Summary report

Notes: There have been 2 designations for this condition: EMA/OD/145/10 S-Nitrosoglutathione, EMA/OD/027/14 Recombinant human alpha-1 microglobulin

2.2.14. - EMA/OD/032/18

Treatment of Duchenne muscular dystrophy

Action: For discussion/adoption

Document(s) tabled:
Draft Summary report

Notes: There have been 27 designations for this condition: See 2.1.10

2.2.15. - EMA/OD/026/18

Treatment of acute myeloid leukaemia

Action: For discussion/adoption

Document(s) tabled:
Draft Summary report

Notes: There have been 54 designations for this condition: EMEA/OD/022/00 Gemtuzumab ozogamicin, EMEA/OD/028/04 Midostaurin, EMEA/OD/056/06 Antisense oligonucleotide 5'-d[P-Thio] (CCCTG CTCCC CCCTG GCTCC)-3' (see comments box for cenersen sodium), EMEA/OD/098/04 Tipifarnib, EMEA/OD/094/04 Histamine dihydrochloride, EMEA/OD/066/05 1,2-bis(methylsulphonyl)-1-(2-chloroethyl)-2-[(methylamino)carbonyl]hydrazine, EMEA/OD/100/05 zosuquidar trihydrochloride, EMEA/OD/004/06 Decitabine, EMEA/OD/049/07 5'-O-(trans-9"-octadecenoyl)-1-ß-D-arabinofuranosyl cytosine, EMEA/OD/087/07 Recombinant human histone H1.3 and recombinant human N-bis-met-histone H1.3, EMEA/OD/085/07 Azacitidine, EMEA/OD/099/07 N-(2-amino-phenyl)-4-[(4-pyridin-3-yl-pyrimidin-2-ylamino)-methyl] benzamide, EMEA/OD/118/07 Ribonucleotide reductase R2 specific phosphorothioate oligonucleotide, EMEA/OD/015/08 Sapacitabine, EMEA/OD/048/08 Daunorubicin (liposomal), EMEA/OD/105/08 N-(5-tert-Butylisoxazol-3-yl)-N'-{4-[7-(2-(morpholin-4-yl)ethoxy) imidazo[2,1-b][1,3]benzothiazol-2-yl]phenyl}urea di-hydrochloride salt, EMEA/OD/028/09 Tosedostat, EMEA/OD/091/09 1-Cyclopropyl-3-[3-(5-morpholin-4-ylmethyl-1H-benzoimidazol-2-yl)-1H-pyrazol-4-yl]-urea, EMEA/OD/147/09 2-methoxymethyl-2-hydroxymethyl-1-azabicyclo[2,2,2]octan-3-one, EMA/OD/044/10 Allogeneic T cells encoding an exogenous TK gene, EMA/OD/094/10 N-[(2S)-2,3-dihydroxypropyl]-3-[(2-fluoro-4-iodophenyl) amino] isonicotinamide hydrochloride, EMA/OD/101/11 Allogeneic human dendritic cells derived from a CD34+ progenitor cell line, EMA/OD/070/11 Liposomal combination of cytarabine and daunorubicin, EMA/OD/158/11 Vosaroxin, EMA/OD/167/12 L-asparaginase encapsulated in erythrocytes, EMA/OD/064/13 trans-N1-((1R,2S)-2-phenylcyclopropyl)cyclohexane-1,4-diamine bis-hydrochloride, EMA/OD/141/13 (2R,3R,4S,5R)-2-(6-amino-9H-purin-9-yl)-5-((((1r,3S)-3-(2-(5-(tert-butyl)-1Hbenzo[d]imidazol-2-yl)ethyl)cyclobutyl)(isopropyl) amino)methyl) tetrahydrofuran-3,4-diol, EMA/OD/181/13 Volasertib, EMA/OD/100/14 4-{[(2R,3S,4R,5S)-4-(4-Chloro-2-fluoro-phenyl)-3-(3-chloro-2-fluoro-phenyl)-4-cyano-5-(2,2-dimethyl-propyl)-pyrrolidine-2-carbonyl]-amino}-3-methoxy-benzoic acid, EMA/OD/061/14 (Z)-3-(3-(3,5-bis(trifluoromethyl)phenyl)-1H-1,2,4-triazol-1-yl)-N'-(pyrazin-2-yl)acrylohydrazide, EMA/OD/103/14 Donor T lymphocytes depleted ex vivo of host alloreactive T cells using photodynamic treatment, EMA/OD/175/14 Allogeneic ex vivo-generated natural killer cells from CD34+ umbilical cord blood progenitor cells, EMA/OD/240/14 Alvocidib, EMA/OD/258/14 Ulocuplumab, EMA/OD/045/15 inecalcitol, EMA/OD/037/15 2-((3-((4-((3-

aminopropyl)amino)butyl)amino)propyl)amino)-N-((5S,5aS,8aR,9R)-9-(4-hydroxy-3,5-dimethoxyphenyl)-8-oxo-5,5a,6,8,8a,9-hexahydrofuro[3',4':6,7]naphtho[2,3-d][1,3]dioxol-5-yl)acetamide, tetrahydrochloride, EMA/OD/089/15 CD33-directed antibody-drug conjugate consisting of an antibody conjugated to a DNA cross-linking pyrrolobenzodiazepine dimer drug, EMA/OD/112/15 Recombinant human interleukin-3 truncated diphtheria toxin fusion protein, EMA/OD/145/15 Humanised monoclonal antibody of the IgG4 kappa isotype targeting CD47, EMA/OD/165/15 Sodium (2R,3S,5R)-5-(4-amino-2-oxo-1,3,5-triazin-1(2H)-yl)-2-(hydroxymethyl)tetrahydrofuran-3-yl ((2R,3S,5R)-5-(2-amino-6-oxo-1H-purin-9(6H)-yl)-3-hydroxytetrahydrofuran-2-yl)methyl phosphate, EMA/OD/144/15 Combretastatin A1-diphosphate, EMA/OD/180/15 Arsenic trioxide, EMA/OD/205/15 Venetoclax, EMA/OD/233/15 Tyr-Met-Phe-Pro-Asn-Ala-Pro-Tyr-Leu, Ser-Gly-Gln-Ala-Tyr-Met-Phe-Pro-Asn-Ala-Pro-Tyr-Leu-Pro-Ser-Cys-Leu-Glu-Ser, Arg-Ser-Asp-Glu-Leu-Val-Arg-His-His-Asn-Met-His-Gln-Arg-Asn-Met-Thr-Lys-Leu and Pro-Gly-Cys-Asn-Lys-Arg-Tyr-Phe-Lys-Leu-Ser-His-Leu-Gln-Met-His-Ser-Arg-Lys-His-Thr-Gly, EMA/OD/253/15 2-methyl-1-[(4-[6-(trifluoromethyl)pyridin-2-yl]-6-{[2-(trifluoromethyl)pyridin-4-yl]amino}-1,3,5-triazin-2-yl)amino]propan-2-ol methanesulfonate, EMA/OD/155/16 P-ethoxy growth factor receptor-bound protein 2 (Grb2) antisense oligonucleotide, EMA/OD/197/16 Ivosidenib, EMA/OD/319/16 225Ac-lintuzumab, EMA/OD/106/17 Glasdegib maleate, EMA/OD/010/17 Sodium (1R, 3R, 4R, 5S)-3-({2-N-acetylamino-2-deoxy-3-O-[(1S)-1-carboxylato-2-cyclohexylethyl]-β-D-galactopyranosyl}oxy)-4-({6-deoxy-α-L-galactopyranosyl}oxy)-5-ethyl-cyclohexan-1-yl-(38-oxo-2,5,8,11,14,17,20,23,26,29,32,35-dodecaoxa-39-azahentetracontan-41-yl) carboxamide, EMA/OD/040/17 Entospletinib, EMA/OD/101/17 Pracinostat, EMA/OD/175/17 Gilteritinib, EMA/OD/193/17 6-{[(1R,2S)-2-aminocyclohexyl]amino}-7-fluoro-4-(1-methyl-1H-pyrazol-4-yl)-1,2-dihydro-3H-pyrrolo[3,4-c]pyridin-3-one monocitrate

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

2.2.16. - EMA/OD/034/18

Treatment of epidermolysis bullosa

Action: For discussion/adoption

Document(s) tabled:
Draft Summary report

Notes: There have been 15 designations for this condition: EMEA/OD/111/05 Bilayer engineered skin composed of keratinocytes from the patient (autologous) and fibroblasts from a donor (allogeneic) embedded in a plasma matrix, EMEA/OD/061/09 Allogeneic human dermal fibroblasts, EMA/OD/120/10 Dry extract from birch bark (DER 0.1-0.2:1), extraction solvent n-heptane 95% (V/V), EMA/OD/145/13 Allantoin, EMA/OD/149/13 Diacerein, EMA/OD/201/13 Recombinant human alpha 1 chain homotrimer of type VII collagen, EMA/OD/197/14 Allogeneic adipose-derived adult mesenchymal stem cells contained in a fibrin-based bioengineered dermis, EMA/OD/218/15 Autologous dermal fibroblasts genetically modified ex vivo with a lentiviral vector containing the human COL7A1 gene, EMA/OD/299/14 Ex-vivo-expanded autologous human keratinocytes containing epidermal stem cells transduced with a COL17A1-encoding retroviral vector, EMA/OD/297/14 Ex-vivo-expanded autologous human keratinocytes containing epidermal stem cells transduced with a LAMB3-encoding retroviral vector, EMA/OD/188/15 Ex-vivo-expanded autologous fibroblasts transduced with lentiviral vector containing the COL7A1 gene, EMA/OD/283/16 Ex-vivo-expanded autologous keratinocytes transduced with retroviral vector containing the COL7A1 gene, EMA/OD/031/17 Asp-Arg-Val-Tyr-Ile-His-Pro, EMA/OD/140/17 Antisense oligonucleotide targeting exon 73 in the COL7A1 gene, EMA/OD/244/17 Genetically modified replication- incompetent herpes simplex virus-1 expressesing collagen VII
Designation withdrawn: EMA/OD/172/10 Human dermal fibroblasts cultured on a bioresorbable polyglactin mesh

2.3. Revision of the COMP opinions

None

2.4. Amendment of existing orphan designations

2.4.1. Interferon beta – EMA/OD/080/07

Faron Pharmaceuticals Limited; Treatment of acute lung injury

Action: For adoption

Document(s) tabled:
Amended draft 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(s) tabled:
OMPD applications - appointment of coord. at the 22-24 May 2018 COMP meeting

2.7. Evaluation on-going

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

Action: For information

Notes: See 7.8.1. Evaluation Ongoing.

3. Requests for protocol assistance with significant benefit question

3.1. Ongoing procedures

3.1.1. -

Treatment of acute myeloid leukaemia

Action: For adoption

3.1.2. -

Treatment of pemphigus

Action: For adoption

3.1.3. -

Treatment of small cell lung cancer

Action: For adoption

3.1.4. -

Treatment of tuberous sclerosis

Action: For adoption

3.1.5. -

Treatment of acute myeloid leukaemia

Action: For adoption

3.1.6. -

Treatment of Fabry disease

Action: For adoption

3.1.7. -

Prevention of oral mucositis in head and neck cancer patients undergoing radiation therapy

Action: For adoption

3.1.8. -

Treatment of congenital hyperinsulinism

Action: For adoption

3.1.9. -

Treatment of naevoid basal-cell carcinoma syndrome (Gorlin syndrome)

Action: For adoption

3.1.10. -

Treatment of multiple myeloma

Action: For adoption

3.2. Finalised letters

3.2.1. -

Treatment of amyotrophic lateral sclerosis

Action: For information

3.2.2. -

Treatment of sickle cell disease

Action: For information

3.2.3. -

Treatment of soft tissue sarcoma

Action: For information

3.2.4. -

Treatment of acute sensorineural hearing loss (acute acoustic trauma, sudden deafness and surgery induced acoustic trauma)

Action: For information

3.2.5. -

Treatment of Cushing's syndrome

Action: For information

3.3. New requests

3.3.1. -

Treatment of bronchiolitis obliterans syndrome

Action: For information

3.3.2. -

Treatment of graft-versus-host disease

Action: For information

3.3.3. -

Treatment of hairy cell leukaemia

Action: For information

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

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

None

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

4.2.1. – daunorubicin/ cytarabine - EMEA/H/C/004282, EMA/OD/070/11, EU/3/11/942

Jazz Pharmaceuticals Ireland Limited; Treatment of adults with high-risk acute myeloid leukaemia (AML)

Action: For adoption, Oral explanation to be held on 22 May 2018 at time 11:00

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

4.2.2. - inotersen – EMEA/H/C/004782, EMA/OD/098/13, EU/3/14/1250

IONIS USA Ltd; Treatment of ATTR amyloidosis

Action: For discussion

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

4.2.3. - metreleptin – EMEA/H/C/004218

Aegerion Pharmaceuticals Limited;

a) Treatment of familial partial lipodystrophy EMA/OD/033/12, EU/3/12/1022

b) Treatment of Barraquer-Simons syndrome EMA/OD/034/12, EU/3/12/1023

c) Treatment of Lawrence syndrome EMA/OD/035/12, EU/3/12/1024

d) Treatment of Berardinelli-Seip syndrome EMA/OD/036/12, EU/3/12/1025

Action: For discussion

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

4.2.4. - vonicog alfa – EMA/OD/055/10, EU/3/10/814, EMEA/H/C/004454

Baxalta Innovations GmbH; Treatment of von Willebrand disease

Action: For discussion

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

4.2.5. - patisiran – EMEA/H/C/004699, EMA/OD/142/10, EU/3/11/857

Alnylam UK Limited; Treatment of familial amyloid polyneuropathy

Action: For discussion

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

4.2.6. - tisagenlecleucel – EMEA/H/C/004090

Novartis Europharm Limited;

a) Treatment of diffuse large B-cell lymphoma EMA/OD/087/16, EU/3/16/1745

b) Treatment of B-lymphoblastic leukaemia/lymphoma EMA/OD/187/13, EU/3/14/1266

Action: For discussion

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

4.3. Revision of the COMP opinions

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

Santen Oy; Treatment of vernal keratoconjunctivitis

Action: For discussion

Document(s) tabled:
EC letter

4.4. Appeal

None

4.5. On-going procedures

Action: For information

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

4.6. Orphan Maintenance Reports

Action: For information

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

5.1. After adoption of CHMP opinion

None

5.2. Prior to adoption of CHMP opinion

5.2.1. Adcetris - Brentuximab vedotin – Type II variation – EMEA/H/C/002455/II/0055

Takeda Pharma A/S; Treatment of Hodgkin lymphoma EMEA/OD/073/08, EU/3/08/596

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

Action: For discussion

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

5.2.2. Coagadex - Human coagulation factor X – Type II variation – EMEA/H/C/003855/II/0007, EMEA/OD/044/07, EU/3/07/471

Bio Products Laboratory Limited; Treatment of hereditary factor X deficiency

CHMP rapporteur: Andrea Laslop

Action: For discussion

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

5.3. Appeal

None

5.4. On-going procedures

Action: For information

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

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

None

7. Organisational, regulatory and methodological matters

7.1. Mandate and organisation of the COMP

7.1.1. COMP Strategic Review & Learning meeting, 26-28 March 2018, Amsterdam, The Netherlands

Action: For adoption

Document(s) tabled:
COMP Strategic Review and Learning Meeting Minutes

7.1.2. Protocol Assistance Working Group (PAWG)

Proposed meeting time on 22 May 2018 at 13:00

Document(s) tabled:
PAWG draft agenda for 22 May 2018 meeting
PAWG draft minutes for 17 April 2018 meeting

7.1.3. Prevalence Working Group

Proposed meeting time on 23 May 2018 at 08:30

7.1.4. Recommendation on criteria for competence and expertise of COMP members / Invitation to propose expert(s) to join the Committee for Orphan Medicinal Products

Action: For information

7.1.5. Conditions Working Group

Proposed meeting time on 24 May 2018 at 08:30

7.2. Coordination with EMA Scientific Committees or CMDh-v

7.2.1. Recommendations on eligibility to PRIME – report from CHMP

Action: For information

Document(s) tabled:
PRIME eligibility requests - list of adopted outcomes April 2018

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

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

Action: For information

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

Action: For information

7.4. Cooperation within the EU regulatory network

7.4.1. European Commission

Guideline on the format and content of applications for designation as orphan medicinal products and on the transfer of designations from one sponsor to another, ENTR/6283/00 Rev 5

Action: For discussion

Document tabled:
EC Guideline_REV 5

7.5. Cooperation with International Regulators

7.5.1. Food and Drug Administration (FDA)

Action: For information

Notes: Monthly teleconference

7.5.2. Japanese Pharmaceuticals and Medical Devices Agency (PMDA)

Action: For information

Notes: Ad hoc basis meeting

7.5.3. The Therapeutic Goods Administration (TGA), Australia

Action: For information

Notes: Ad hoc basis meeting

7.5.4. Health Canada

Action: For information

Notes: Ad hoc basis meeting

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

None

7.7. COMP work plan

None

7.8. Planning and reporting

7.8.1. List of all applications submitted/expected and the COMP coordinatorship distribution of valid applications submitted in 2018

Action: For information

7.8.2. Overview of orphan marketing authorisations/applications

Action: For information

8. Any other business

None

9. Explanatory notes

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

Abbreviations / Acronyms

CHMP: Committee for Medicinal Product for Human Use

COMP: Committee for Orphan Medicinal Products

EC: European Commission

OD: Orphan Designation

PA: Protocol Assistance

PDCO: Paediatric Committee

PRAC: Pharmacovigilance and Risk Assessment Committee

SA: Scientific Advice

SAWP: Scientific Advice Working Party

Orphan Designation *(section 2 Applications for orphan medicinal product designation)*

The orphan designation is the appellation given to certain medicinal products under development that are intended to diagnose, prevent or treat rare conditions when they meet a pre-defined set of criteria foreseen in the legislation. Medicinal products which get the orphan status benefit from several incentives (fee reductions for regulatory procedures (including protocol assistance), national incentives for research and development, 10-year market exclusivity) aiming at stimulating the development and availability of treatments for patients suffering from rare diseases.

Orphan Designations are granted by Decisions of the European Commission based on opinions from the COMP. Orphan designated medicinal products are entered in the Community Register of Orphan Medicinal Products.

Protocol Assistance *(section 3 Requests for protocol assistance with significant benefit question)*

The protocol assistance is the help provided by the Agency to the sponsor of an orphan medicinal product, on the conduct of the various tests and trials necessary to demonstrate the quality, safety and efficacy of the medicinal product in view of the submission of an application for marketing authorisation.

Sponsor

Any legal or physical person, established in the Community, seeking to obtain or having obtained the designation of a medicinal product as an orphan medicinal product.

Maintenance of Orphan Designation *(section 4 Review of orphan designation for orphan medicinal products for marketing authorisation).*

At the time of marketing authorisation, the COMP will check if all criteria for orphan designation are still met. The designated orphan medicinal product should be removed from the Community Register of Orphan Medicinal Products if it is established that the criteria laid down in the legislation are no longer met.

More detailed information on the above terms can be found on the EMA website: www.ema.europa.eu/