29 September 2017
EMA/COMP/606831/2017
Inspections, Human Medicines Pharmacovigilance and Committees

Committee for Orphan Medicinal Products (COMP)

Draft agenda for the meeting on 03-05 October 2017

Chair: Bruno Sepodes – Vice-Chair: Lesley Greene

03 October 2017, 09:00-19:30, room 2F

04 October 2017, 08:30-19:30, room 2F

05 October 2017, 08:30-17:00, 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 03-05 October 2017. See October 2017 COMP minutes (to be published post 30-31 October 2017 COMP meeting).

1.2. Adoption of agenda

COMP agenda for 03-05 October 2017.

1.3. Adoption of the minutes

COMP minutes for 05-07 September 2017.

2. Applications for orphan medicinal product designation

2.1. For opinion

2.1.1. - EMA/OD/119/17

Treatment of gastrointestinal stromal tumours

Action: For adoption, Oral explanation to be held on 03 October 2017 at 09:30

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

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

Designation withdrawn: EMEA/OD/093/06 Nilotinib hydrochloride monohydrate

2.1.2. - EMA/OD/120/17

Treatment of subarachnoid haemorrhage (SAH)

Action: For adoption, Oral explanation to be held on 03 October 2017 at 16:30

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

2.1.3. - EMA/OD/127/17

Treatment of subarachnoid hemorrhage

Action: For adoption, Oral explanation to be held on 03 October 2017 at 17:30

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

2.1.4. - EMA/OD/105/17

Treatment in solid organ transplantation

Action: For adoption, Oral explanation to be held on 04 October 2017 at 09:30

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

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

2.1.5. - EMA/OD/110/17

Treatment of focal segmental glomerulosclerosis

Action: For information

Document(s) tabled:
Withdrawal request of 18 September 2017

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

Designation withdrawn: EMA/OD/146/10 Fresolimumab

2.1.6. - EMA/OD/107/17

Treatment of idiopathic pulmonary fibrosis

Action: For information

Document(s) tabled:
Withdrawal request of 18 September 2017

Notes:
There have been 12 designations for this condition: EMEA/OD/052/04 Pirfenidone, EMEA/OD/054/07 Interferon gamma, EMEA/OD/104/09 Macitentan, EMA/OD/079/10 2-(2-chlorophenyl)-4-[3-(dimethylamino)phenyl]-5-methyl-1H-pyrazolo[4,3-C]pyridine-3,6(2H,5H)-dione, EMA/OD/091/11 4-[[9-[(3S)-tetrahydro-3-furanyl]-8-[(2,4,6-trifluorophenyl)amino]-9H-purin-2-yl]amino]-trans-cyclohexanol, EMA/OD/048/12 Recombinant human pentraxin-2, EMA/OD/186/12 nintedanib, EMA/OD/051/14 Humanised anti-alpha v beta 6 monoclonal antibody, EMA/OD/130/14 1-(6-benzothiazolylsulfonyl)-5-chloro-1H-indole-2-butanoic acid, EMA/OD/072/15 3-pentylbenzeneacetic acid sodium salt, EMA/OD/046/16 3-[4-(1H-imidazol-1-ylmethyl)phenyl]-5-(2-methylpropyl)thiophene-2-[(N-butyloxylcarbamate)-sulphonamide] sodium salt, EMA/OD/088/16 2-((2-ethyl-6-(4-(2-(3-

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

Designations withdrawn: EMEA/OD/002/05 Interferon gamma, EMEA/OD/033/04 Heparin-Sodium, EMEA/OD/075/04 Acetylcysteine, EMEA/OD/105/07 Recombinant human monoclonal antibody against transforming growth factor beta-1, 2 and 3, EMEA/OD/027/08 Bosentan, EMA/OD/029/10 Ambrisentan, EMA/OD/111/12 Tralokinumab

2.1.7. - EMA/OD/117/17

Treatment of beta-thalassaemia intermedia and major

Action: For information

Document(s) tabled:
Withdrawal request of 26 September 2017

Notes:
There have been 6 designations for this condition: EMEA/OD/098/08 2,2-dimethylbutyric acid, sodium salt, EMEA/OD/111/08 Autologous haematopoietic stem cells transduced with lentiviral vector encoding the human beta-globin gene, EMA/OD/047/14 Recombinant fusion protein consisting of a modified form of the extracellular domain of human activin receptor IIB linked to the human IgG1 Fc domain, EMA/OD/142/15 Sirolimus, EMA/OD/146/12 Autologous CD34+ haematopoietic stem cells transduced with lentiviral vector encoding the human βA-T87Q-globin gene, EMA/OD/189/14 Benserazide hydrochloride

2.1.8. - EMA/OD/121/17

Treatment of peripheral T-cell lymphoma

Action: For adoption, Oral explanation to be held on 04 October 2017 at 14:00

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

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

2.1.9. - EMA/OD/113/17

Treatment of haemophilia B

Action: For adoption, Oral explanation to be held on 04 October 2017 at 15:00

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

Notes:
There have been 8 designations for this condition: EMEA/OD/117/09 Recombinant fusion protein linking human coagulation factor IX with human albumin, EMA/OD/133/10 Recombinant fusion protein linking human coagulation factor VIIa with human albumin, EMA/OD/090/11 Adeno-associated viral vector containing the human factor IX gene, EMA/OD/041/14 Synthetic double-stranded siRNA oligonucleotide directed against antithrombin mRNA that is covalently linked to a ligand containing three N-

acetylgalactosamine residues, EMA/OD/073/14 Recombinant factor VIIa modified with three terminal repeats derived from the β chain of human chorionic gonadotropin, EMA/OD/003/15 Adeno-associated viral vector containing the human factor IX gene, EMA/OD/172/15 Adeno-associated virus viral vector serotype rh10 encoding containing the human factor IX gene, EMA/OD/018/17 Recombinant human factor IX protein modified with three point mutations

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

2.1.10. - EMA/OD/115/17

Treatment of Prader-Willi-Syndrome (PWS)

Action: For adoption, Oral explanation to be held on 04 October 2017 at 16:30

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

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

Designations withdrawn: EMA/OD/023/14 Beloranib

2.1.11. - EMA/OD/102/17

Treatment of sickle cell disease

Action: For adoption, Oral explanation to be held on 04 Ocotober 2017 at 17:30

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

Notes:
There have been 12 designations for this condition: EMEA/OD/017/05 Extract of Sorghum bicolour leaf, Pterocarpus osun stem, Piper guineense seed and Caryophylli flower, EMEA/OD/107/08 2,2-dimethylbutyric acid, sodium salt, EMEA/OD/075/09 Pegylated carboxyhaemoglobin, EMA/OD/016/12 Levoglutamide, EMA/OD/040/12 Human Erythrocytes encapsulating Inositol Hexaphosphate, EMA/OD/026/12 Humanised monoclonal antibody targeting P-selectin, EMA/OD/084/13 (1R,3R,4R,5S)-3-O-[2-O-benzoyl-3-O-(sodium(2S)-3-cyclohexyl-propanoate-2-yl)-β-D-galactopyranosyl]-4-O-(α-L-fucopyranosyl)-5-orothylamido-cyclohexane-1-carboxylic acid (ethyl-2-amidyl-ethyloxy-2-acetyl-(8-amino-1,3,6-naphthalene-tris sodium sulfonate) amide, EMA/OD/184/13 Autologous CD34+ haematopoietic stem cells transduced with lentiviral vector encoding the human beta A-T87Q-globin gene, EMA/OD/210/14 Sevufparin sodium, EMA/OD/187/16 2-hydroxy-6-((2-(1-isopropyl-1H-pyrazol-5-yl)pyridin-3-yl) methoxy)benzaldehyde, EMA/OD/144/16 Synthetic human hepcidin, EMA/OD/008/17 Decitabine and tetrahydrouridine

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

2.1.12. - EMA/OD/116/17

Treatment of haemophilia B

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

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

Notes:
There have been 8 designations for this condition: EMEA/OD/117/09 Recombinant fusion protein linking human coagulation factor IX with human albumin, EMA/OD/133/10 Recombinant fusion protein linking human coagulation factor VIIa with human albumin, EMA/OD/090/11 Adeno-associated viral vector containing the human factor IX gene, EMA/OD/041/14 Synthetic double-stranded siRNA oligonucleotide directed against antithrombin mRNA that is covalently linked to a ligand containing three N-acetylgalactosamine residues, EMA/OD/073/14 Recombinant factor VIIa modified with three terminal repeats derived from the β chain of human chorionic gonadotropin, EMA/OD/003/15 Adeno-associated viral vector containing the human factor IX gene, EMA/OD/172/15 Adeno-associated virus viral vector serotype rh10 encoding containing the human factor IX gene, EMA/OD/018/17 Recombinant human factor IX protein modified with three point mutations

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

2.1.13. - EMA/OD/126/17

Treatment of microvillus inclusion disease

Action: For adoption, Oral explanation to be held on 05 October 2017 at 11:00

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

2.1.14. - EMA/OD/109/17

Treatment of chronic thromboembolic pulmonary hypertension (CTEPH)

Action: For adoption, Oral explanation to be held on 05 October 2017 at 12:00

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

2.1.15. - EMA/OD/067/17

Treatment of pancreatic cancer

Action: For information

Document(s) tabled:
Withdrawal request of 27 September 2017

Notes:

There have been 37 designations for this condition: EMEA/OD/055/02 G17(9) gastrin-Diphtheria Toxoid Conjugate, EMEA/OD/068/02 Rubitecan, EMEA/OD/009/05 Bovine bile extract, EMEA/OD/028/05 4-imino-1, 3-diazobicyclo-[3.1.0]-hexan-2-one, EMEA/OD/063/06 Paclitaxel (liposomal), EMEA/OD/026/06 Human telomerase reverse transcriptase peptide (611-626), EMEA/OD/103/06 Cisplatin (liposomal), EMEA/OD/100/08 L-asparaginase encapsulated in erythrocytes, EMEA/OD/006/08 Nimotuzumab, EMEA/OD/080/08 Yttrium (90Y)-DOTA-radiolabelled humanized monoclonal antibody against mucin 1, EMEA/OD/101/08 S-[2,3-bispalmitoyloxy-(2R)-propyl]-cysteinyl-GNNDESNISFKEK, EMEA/OD/030/09 Trabedersen, EMEA/OD/105/09 Brivudine, EMEA/OD/069/09 N-[(2S)-2,3-dihydroxypropyl]-3-[(2-fluoro-4-iodophenyl)amino]isonicotinamide hydrochloride, EMEA/OD/063/09 Masitinib mesilate, EMA/OD/135/10 Glufosfamide, EMA/OD/150/10 Salirasib, EMA/OD/007/11 Mixture of seven synthetic fragments consisting of p21 RAS peptides, EMA/OD/008/11 Genetically modified human adenovirus encoding human PH20 hyaluronidase, EMA/OD/051/11 Nanoliposomal irinotecan, EMA/OD/065/12 Mixture of two allogeneic human pancreatic cancer cell lines stably transduced with a retroviral vector encoding the murine alpha-(1,3)-galactosyltransferase gene, EMA/OD/037/13 (1-methyl-2-nitro-1H-imidazole-5-yl)methyl N,N'-bis(2-bromoethyl)diamidophosphate, EMA/OD/071/13 Chimeric monoclonal antibody against claudin-18 splice variant 2, EMA/OD/164/13 Cysteamine bitartrate, EMA/OD/081/14 Immunoglobulin G1, anti-(human tumour-associated calcium signal transducer 2)(human-Mus musculus monoclonal hRS7 heavy chain), disulfide with human-Mus musculus monoclonal hRS7-chain, dimer, hexakis(thioether) with (4S)-4-[[[[4-[[(2S)-2-(4-aminobutyl)-2-[[2-[2-[[26-[4-[[[[4-[(3-mercapto-2,5-dioxo-1-pyrrolidinyl)methyl]cyclohexyl]carbonyl]amino]methyl]-1H-1,2,3-triazol-1-yl]-3,6,9,12,15,18,21,24-octaoxahexacos-1-yl]amino]-2-oxoethoxy]acetyl]amino]-1-oxoethyl]amino]phenyl]methoxy]carbonyl]oxy]-4,11-diethyl-9-hydroxy-1H-pyrano[3',4':6,7]indolizino[1,2-b]quinoline-3,14(4H,12H)-dione, EMA/OD/085/14 [5-Amino-1-(4-fluoro-phenyl)-1H-pyrazol-4-yl]-[3-(2,3-dihydroxy-propoxy)-phenyl]-methanone, EMA/OD/187/14 Herpes simplex type 1 virus containing cellular B-myb gene as tumour-specific promoter, EMA/OD/143/14 Heat-killed Mycobacterium obuense (whole cell), EMA/OD/173/14 Pegylated recombinant human hyaluronidase PH20, EMA/OD/302/14 Human reovirus type 3 Dearing strain, EMA/OD/034/15 Modified adenovirus serotype 5/35 containing a CMV promoter-driven transgene cassette with the human transgenes for a membrane-bound CD40 ligand (TMZ-CD40L) and full length 4-1BBL, EMA/OD/168/15 Live attenuated Listeria monocytogenes delta actA/delta inlB strain expressing human mesothelin, EMA/OD/169/15 Two allogenic irradiated pancreatic tumour cell lines, EMA/OD/193/16 Pegylated recombinant human interleukin-10, EMA/OD/241/16 Antroquinonol, EMA/OD/273/16 Humanised IgG4 monoclonal antibody to the human toll-like receptor type 2, EMA/OD/078/17 Sodium 2-hydroxylinoleate

Designations withdrawn: EMEA/OD/070/02 Iodine (131I) Anti-CEA sheep-human chimeric monoclonal antibody, EMEA/OD/040/04 Deuterium oxide, EMEA/OD/097/05 26 base single stranded phosphodiester DNA oligonucleotide, EMEA/OD/111/07 Chimeric antibody to mesothelin, EMEA/OD/067/09 5'-O-(trans-9''-octadecenoyl)-1-beta-D-2'deoxy-2',2'-difluorocytidine, EMA/OD/087/10 Nanoparticle albumin-bound paclitaxel, EMA/OD/007/12 Polyinosine-polycytidylic acid coupled with the polycationic polyethyleneimine, EMA/OD/145/12 Recombinant human monoclonal antibody of the IgG1 kappa class against prostate stem cell antigen

2.2. For discussion / preparation for an opinion

2.2.1. - EMA/OD/142/17

Treatment of systemic sclerosis

Action: For discussion/adoption

Document(s) tabled:
Draft Summary report

Notes:
There have been 16 designations for this condition: EMEA/OD/032/01 Halofuginone hydrobromide, EMEA/OD/035/05 Peptide 144 TGF-beta1-inhibitor (TSLDASIIWAMMQN), EMEA/OD/079/08 Type I native bovine skin collagen, EMEA/OD/106/08 Treprostinil diethanolamine, EMA/OD/095/10 Paquinimod, EMA/OD/143/12 2-[4-Methoxy-3-(2-m-tolyl-ethoxy)-benzoylamino]-indan-2-carboxylic acid, EMA/OD/153/12 Terguride, EMA/OD/044/14 Riociguat, EMA/OD/129/14 1-(6-benzothiazolylsulfonyl)-5-chloro-1H-indole-2-butanoic acid, EMA/OD/148/14 Humanized IgG1 monoclonal antibody against human eotaxin-2, EMA/OD/225/14 Nitroglycerin, EMA/OD/296/14 Autologous adipose tissue-derived stromal vascular fraction cells, EMA/OD/105/15 2-(2-chlorophenyl)-4-[3-(dimethylamino)phenyl]-5-methyl-1H-pyrazolo[4,3-C]pyridine-3,6(2H,5H)-dione, EMA/OD/257/15 Autologous stromal vascular cell fraction from adipose tissue, EMA/OD/095/16 Nintedanib, EMA/OD/243/16 (6aR, 10aR)-3-(1',1'-dimethylheptyl)-delta-8-tetrahydro-cannabinol-9-carboxylic acid

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

2.2.2. - EMA/OD/136/17

Treatment of amyotrophic lateral sclerosis

Action: For discussion/adoption

Document(s) tabled:
Draft Summary report

Notes:
There have been 21 designations for this condition: EMEA/OD/053/06 Arimoclomol, EMEA/OD/102/07 Filgrastim, EMEA/OD/096/08 (6R)-4,5,6,7-tetrahydro-N6-propyl-2,6-benzothiazole-diamine dihydrochloride monohydrate, EMEA/OD/108/09 Recombinant human vascular endothelial growth factor, EMA/OD/043/11 Smilagenin, EMA/OD/106/11 S[+] apomorphine, EMA/OD/138/11 6-ethynyl-1-(pentan-3-yl)-1H-imidazo[4,5-b]pyrazin-2(3H)-one, EMA/OD/011/13 Autologous bone marrow-derived mesenchymal stromal cells secreting neurotrophic factors, EMA/OD/023/13 Sodium chlorite, EMA/OD/044/13 Allogeneic motor neuron progenitor cells derived from human embryonic stem cells, EMA/OD/184/14 Edaravone, EMA/OD/283/14 Enoxacin, EMA/OD/032/15 Edaravone, EMA/OD/051/15 Hydrocinnamate-[Orn-Pro-dCha-Trp-Arg]acetate, EMA/OD/011/16 H-Phe-Ser-Arg-Tyr-Ala-Arg-OH-acetate, EMA/OD/241/15 Recombinant human cerebral dopamine neurotrophic factor, EMA/OD/081/16 Masitinib mesilate, EMA/OD/120/16 Synthetic ribonucleic acid oligonucleotide directed against superoxide dismutase 1 messenger ribonucleic acid,

EMA/OD/182/16 Ibudilast, EMA/OD/242/16 Tauroursodeoxycholic acid, EMA/OD/030/17 Recombinant human antibody directed against misfolded human superoxide dismutase 1

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

2.2.3. - EMA/OD/132/17

Treatment of mitochondrial encephalomyopathy, lactic acidosis and stroke-like episodes

Action: For discussion/adoption

Document(s) tabled:
Draft Summary report

Notes:
There has been 1 designation for this condition: EMA/OD/085/15 (S)-6-hydroxy-2,5,7,8-tetramethyl-N-((R)-piperidin-3-yl)chroman-2-carboxamide hydrochloride

2.2.4. - EMA/OD/118/17

Treatment of pancreatic cancer

Action: For discussion/adoption

Document(s) tabled:
Draft Summary report

Notes:
There have been 37 designations for this condition: Please see 2.1.15.

2.2.5. - EMA/OD/135/17

Treatment of cerebral cavernous malformation

Action: For discussion/adoption

Document(s) tabled:
Draft Summary report

2.2.6. - EMA/OD/139/17

Treatment of spinal cord injury

Action: For discussion/adoption

Document(s) tabled:
Draft Summary report

Notes:
There have been 5 designations for this condition: EMEA/OD/082/07 3-methoxy-pregnenolone, EMEA/OD/059/08 Recombinant human monoclonal antibody to human Nogo-A protein of the IgG4/kappa class, EMEA/OD/042/08 Filgrastim, EMA/OD/119/13 synthetic

12 amino acids peptide designed after subcommissural organ-spondin, EMA/OD/325/16 Oxymetazoline hydrochloride

Designation withdrawn: EMEA/OD/041/08 Autologous urothelial and smooth muscle cells

2.2.7. - EMA/OD/140/17

Treatment of epidermolysis bullosa

Action: For discussion/adoption

Document(s) tabled:
Draft Summary report

Notes:
There have been 13 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

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

2.2.8. - EMA/OD/130/17

Treatment of hepatocellular carcinoma

Action: For discussion/adoption

Document(s) tabled:
Draft Summary report

Notes:
There have been 21 designations for this condition: EMEA/OD/015/02 Thymalfasin, EMEA/OD/087/04 Pegylated arginine deiminase, EMEA/OD/048/04 Doxorubicine polyisohexylcyanoacrylate nanoparticles, EMEA/OD/018/05 Nemorubicin hydrochloride, EMEA/OD/109/05 Sorafenib tosylate, EMEA/OD/070/09 NGR-human tumour necrosis factor, EMEA/OD/076/09 Vaccinia GM-CSF/TK-deactivated virus, EMA/OD/065/10 (S)-10-[(dimethylamino)methyl]-4-ethyl-9-hydroxy-4-O-[alpha-(2", 4", 5", 7"-tetranitro-9"-fluorenylideneaminooxy)propionyl]-1H-pyrano[3', 4', 6', 7']indolizino[1,2-beta]-quinoline-3, 14-(4H, 12H)-dione, hydrochloride, EMA/OD/096/10 Doxorubicin hydrochloride (in heat-

sensitive liposomes), EMA/OD/170/10 Sulfonated monophosphorylated mannose oligosaccharide, EMA/OD/003/11 Peretinoin, EMA/OD/045/11 Resminostat, EMA/OD/159/12 4-[2-(6-methylpyridin-2-yl)-5,6-dihydro-4H-pyrrolo[1,2-b]pyrazol-3-yl]-quinoline-6-carboxamide monohydrate, EMA/OD/115/13 Tivantinib, EMA/OD/160/14 Diaspirin cross-linked haemoglobin, EMA/OD/287/14 Lenvatinib, EMA/OD/087/15 2-(2-phenylvinyl)-4-[4-methylpiperazin-1-yl)]-6-(5-methyl-2H-pyrazol-3-yl-amino)-pyrimidine L(+) tartrate salt, EMA/OD/118/15 2-chloro-N6-(3-iodobenzyl)adenosine-5'-N-methyluronamide, EMA/OD/072/16 Mifamurtide, EMA/OD/052/17 N-{2-[(6-{[(2,6-dichloro-3,5-dimethoxyphenyl)carbamoyl](methyl)amino}pyrimidin-4-yl)amino]-5-(4-ethylpiperazin-1-yl)phenyl}prop-2-enamide, EMA/OD/038/17 Tirapazamine

Designations withdrawn: EMEA/OD/013/01 Seocalcitol, EMEA/OD/026/02 Doxorubicin carbon/iron magnetically targeted microparticles, EMEA/OD/032/03 Nolatrexed, EMEA/OD/090/07 N-[4-(3-amino-1H-indazol-4 yl)phenyl]-N'-(2-fluoro-5-methylphenyl) urea, EMEA/OD/046/07 4-[3,5-bis(trimethylsilyl)benzamido] benzoic acid, EMA/OD/075/11 Brivanib alaninate, EMA/OD/031/12 Ramucirumab

2.2.9. - EMA/OD/143/17

Treatment of myotonic disorders

Action: For discussion/adoption

Document(s) tabled:
Draft Summary report

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

2.2.10. - EMA/OD/138/17

Treatment of Fabry disease

Action: For discussion/adoption

Document(s) tabled:
Draft Summary report

Notes:
There have been 4 designations for this condition: EMEA/OD/105/05 1-deoxygalactonojirimycin hydrochloride, EMA/OD/042/12 N-Butyldeoxygalactonojirimycin, EMA/OD/052/14 (3S)-1-azabicyclo[2.2.2]oct-3-yl {2-[2-(4-fluorophenyl)-1,3-thiazol-4-yl]propan-2-yl}carbamate, EMA/OD/277/16 Adeno-associated viral vector serotype 8 containing the human alpha-galactosidase A gene

2.2.11. - EMA/OD/134/17

Treatment of mucopolysaccharidosis type II (Hunter syndrome)

Action: For discussion/adoption

Document(s) tabled:
Draft Summary report

Notes:
There has been 1 designation for this condition: EMA/OD/076/15 Adeno-associated viral vector serotype 9 containing the human iduronate-2-sulfatase gene

2.2.12. - EMA/OD/133/17

Treatment of Duchenne muscular dystrophy

Action: For discussion/adoption

Document(s) tabled:
Draft Summary report

Notes:
There have been 26 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/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, 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

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

2.2.13. - EMA/OD/128/17

Treatment of Allan-Herndon-Dudley-Syndrome

Action: For discussion/adoption

Document(s) tabled:
Draft Summary report

Notes:
There has been 1 designation for this condition: EMA/OD/089/13 3,5-diiodothyropropionic acid

2.3. Revision of the COMP opinions

None

2.4. Amendment of existing orphan designations

None

2.5. Appeal

None

2.6. Nominations

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

Action: For adoption

Document(s) tabled:
OMPD applications - appointment of coord. at the 03-05 October 2017 COMP meeting

2.7. Evaluation on-going

Twenty 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

None

3.2. Finalised letters

3.2.1. -

Treatment of paroxysmal nocturnal haemoglobinuria

Action: For information

3.2.2. -

Treatment of congenital factor VII deficiency

Action: For information

3.2.3. -

Treatment of myelodysplastic syndromes

Action: For information

3.2.4. -

Treatment in solid organ transplantation

Action: For information

3.3. New requests

3.3.1. -

Treatment of spinal muscular atrophy

Action: For information

3.3.2. -

Treatment of plasma cell myeloma

Action: For information

3.3.3. -

Treatment of sickle cell disease

Action: For information

3.3.4. -

Treatment of chronic lymphocytic leukaemia

Action: For information

3.3.5. -

Treatment of idiopathic pulmonary fibrosis

Action: For information

3.3.6. -

Treatment of small cell lung cancer

Action: For information

3.3.7. -

Treatment of ornithine transcarbamylase deficiency

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

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

Tesaro UK Limited; Treatment of ovarian cancer

Action: For adoption, Oral explanation to be held on 03 October 2017 at time 12:00

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

Notes:
Status of the procedure at the CHMP: CHMP adopted opinion in September 2017

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

4.2.1. Lenvima - Lenvatinib - EMA/OD/287/14, EU/3/15/1460, EMEA/H/C/003727/II/0011/G

Eisai Ltd; Treatment of hepatocellular carcinoma

CHMP rapporteur: Bart Van der Schueren; CHMP co-rapporteur: Robert James Hemmings

Action: For information

4.3. Appeal

None

4.4. On-going procedures

Action: For information

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

4.5. Public Summary of Opinions

Action: For information

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

5.1. After adoption of CHMP opinion

None

5.2. Prior to adoption of CHMP opinion

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

Eisai Ltd; Treatment of Lennox-Gastaut syndrome

CHMP rapporteur: Alexandre Moreau

Action: For discussion

5.2.2. Translarna – Ataluren - Type II variation - EMEA/OD/106/04, EU/3/05/278, EMEA/H/C/002720

PTC Therapeutics International Limited; Treatment of duchenne muscular dystrophy

CHMP rapporteur: Johann Lodewijk Hillege

Action: For discussion

5.3. Appeal

None

5.4. On-going procedures

Action: For information

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

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

None

7. Organisational, regulatory and methodological matters

7.1. Mandate and organisation of the COMP

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

Action: For information

Document(s) tabled:
Presentations

7.1.2. Protocol Assistance Working Group (PAWG)

Proposed meeting time on 03 October 2017 at 13:00

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

7.1.3. Non-Clinical Working Group

Proposed meeting time on 04 October 2017 2017 at 08:30

7.1.4. Condition Working Group

Proposed meeting time on 05 October 2017 at 08:30

7.2. Coordination with EMA Scientific Committees or CMDh-v

7.2.1. Cell based ATMPs-Orphan Nomenclature

Action: For information

Document(s) tabled:
Presentation Cell based Orphan-ATMPs Nomenclature

7.2.2. Recommendations on eligibility to PRIME – report from CHMP

Action: For information

Document(s) tabled:
PRIME eligibility requests - list of adopted outcomes September 2017

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

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

PCWP/HCPWP joint meeting - 27-28 June 2017

Action: For information

Document(s) tabled:

Minutes PCWP/HCPWP joint meeting – 27-28 June 2017 (EMA/355452/2017)

7.3.2. Scientific Advice Working Party (SAWP)

Action: For information

7.4. Cooperation within the EU regulatory network

7.4.1. European Commission

None

7.5. Cooperation with International Regulators

7.5.1. Food and Drug Administration (FDA)

Action: For information

Notes:
Monthly teleconference

7.5.2. Japanese Pharmaceuticals and Medical Devices Agency (PMDA)

Action: For information

Notes:
Ad hoc basis meeting

7.5.3. The Therapeutic Goods Administration (TGA), Australia

Action: For information

Notes:
Ad hoc basis meeting

7.5.4. Health Canada

Action: For information

Notes:
Ad hoc basis meeting

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

None

7.7. COMP work plan

Action: For information

Document(s) tabled:
COMP Work Plan 2017

7.8. Planning and reporting

7.8.1. List of all applications submitted/expected and the COMP coordinatorship distribution of valid applications submitted in 2017

Action: For information

7.8.2. Overview of orphan marketing authorisations/applications

Action: For information

8. Any other business

8.1. S-REPS: a new way of supporting COMP procedures with a CRM (Customer Relationship Management software)

Action: For information

8.2. Publication of review of orphan criteria report

Action: For information

8.3. Preparedness of the system and capacity increase

Action: For discussion

8.4. COMP Workshop on Prevalence

The workshop will take place on 4 December 2017 at the EMA.

Action: For information

Document(s) tabled:
Draft agenda

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/