24 October 2017
EMA/COMP/663531/2017
Inspections, Human Medicines Pharmacovigilance and Committees

Committee for Orphan Medicinal Products (COMP)

Draft agenda for the meeting on 30-31 October 2017

Chair: Bruno Sepodes – Vice-Chair: Lesley Greene

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

31 October 2017, 08:30-16:30, room 2F

Health and safety information

In accordance with the Agency's health and safety policy, delegates are to be briefed on health, safety and emergency information and procedures prior to the start of the meeting.

Disclaimers

Some of the information contained in this agenda is considered commercially confidential or sensitive and therefore not disclosed. With regard to intended therapeutic indications or procedure scopes listed against products, it must be noted that these may not reflect the full wording proposed by applicants and may also vary during the course of the review. Additional details on some of these procedures will be published in the COMP meeting reports once the procedures are finalised.

Of note, this agenda is a working document primarily designed for COMP members and the work the Committee undertakes.

Note on access to documents

Some documents mentioned in the agenda cannot be released at present following a request for access to documents within the framework of Regulation (EC) No 1049/2001 as they are subject to on-going procedures for which a final decision has not yet been adopted. They will become public when adopted or considered public according to the principles stated in the Agency policy on access to documents (EMA/127362/2006).

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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 30-31 October 2017. See 30-31 October 2017 COMP minutes (to be published post 5-7 December 2017 COMP meeting).

1.2. Adoption of agenda

COMP agenda for 30-31 October 2017.

1.3. Adoption of the minutes

COMP minutes for 03-05 October 2017.

2. Applications for orphan medicinal product designation

2.1. For opinion

2.1.1. - EMA/OD/132/17

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

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

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

2.1.2. - EMA/OD/143/17

Treatment of myotonic disorders

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

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

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

2.1.3. - EMA/OD/135/17

Treatment of cerebral cavernous malformation

Action: For adoption, Oral explanation to be held on 30 October 2017 at 17:00

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

2.1.4. - EMA/OD/138/17

Treatment of Fabry disease

Action: For adoption, Oral explanation to be held on 31 October 2017 at 11:00

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

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

2.1.5. - EMA/OD/139/17

Treatment of spinal cord injury

Action: For adoption, Oral explanation to be held on 31 October 2017 at 12:00

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

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

2.1.6. - EMA/OD/130/17

Treatment of hepatocellular carcinoma

Action: For information

Document(s) tabled:
Withdrawal request of 10 October 2017

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. For discussion / preparation for an opinion

2.2.1. - EMA/OD/156/17

Treatment of paroxysmal nocturnal hemoglobinuria

Action: For discussion/adoption

Document(s) tabled:
Draft Summary report

Notes: There have been 6 designations for this condition: 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/077/16 Recombinant protein derived from the saliva of the Ornithodoros moubata tick, EMA/OD/246/15 Fc- and CDR-modified humanised monoclonal antibody against C5, EMA/OD/107/16 Synthetic-15-amino acid macrocyclic peptide acylated with a polyethyleneglycol palmitoylated linker, EMEA/OD/042/03 Eculizumab, EMA/OD/004/17 Poly(oxy-1,2-ethanediyl), .alpha.-hydro-.omega.-hydroxy-,15,15'-diester with N-acetyl-L-isoleucyl-L-cysteinyl-L-valyl-1-methyl-L-tryptophyl-L-glutaminyl-L-.alpha.-aspartyl-L-tryptophylglycyl-L-alanyl-L-histidyl-L-arginyl-L-cysteinyl-L-threonyl-2-[2-(2-aminoethoxy)ethoxy]acetyl-N6-carboxy-L-lysinamide cyclic (2.fwdarw.12)-(disulfide); where two identical synthetic peptide domains are covalently linked at the ends of the polyethylene glycol chain
Designation withdrawn: EMEA/OD/016/02 Myristolated-peptidyl-recombinant Human CD59

2.2.2. - EMA/OD/158/17

Treatment of GM2 Gangliosidosis

Action: For discussion/adoption

Document(s) tabled:
Draft Summary report

2.2.3. - EMA/OD/151/17

Treatment of Huntington's disease

Action: For discussion/adoption

Document(s) tabled:
Draft Summary report

Notes: There have been 6 designations for this condition: EMEA/OD/021/00 Ethyl Eicosapentaenoate, EMEA/OD/011/05 4-[3-(methylsulfonyl)phenyl]-1-propylpiperidine x HCl, EMEA/OD/066/09 6-chloro-2,3,4,9-tetrahydro-1H-carbazole-1-carboxamide, EMEA/OD/095/09 Lithium citrate tetrahydrate (in reverse- micelle formulation), EMA/OD/192/14 2'-O-methyl phosphorothioate RNA oligonucleotide, 5' m5CUGm5CUGm5CUGm5CUGm5CUGm5CUGm5CUG-3', EMA/OD/066/16 2-[4-(1-methyl-4-pyridin-4-yl-1H-pyrazol-3-yl)-phenoxymethyl]-quinoline succinic acid
Designation withdrawn: EMEA/OD/061/08 2,3,4,5 tetrahydro-2,8-dimethyl-5-[2-(6-methyl-3-pyridinyl)ethyl]-1H-pyrido[4,3-b]indole dihydrochloride

2.2.4. - EMA/OD/167/17

Treatment of mucopolysaccharidosis type I

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

2.2.5. - EMA/OD/168/17

Treatment of mucopolysaccharidosis type II

Action: For discussion/adoption

Document(s) tabled:
Draft Summary report

Notes: There have been 3 designations for this condition: EMA/OD/076/15 Adeno-associated viral vector serotype 9 containing the human iduronate-2-sulfatase gene, EMA/OD/091/13 Recombinant human insulin receptor monoclonal antibody-fused iduronate 2-sulfatase, EMEA/OD/056/01 Iduronate-2-sulfatase

2.2.6. - EMA/OD/162/17

Treatment of Leber congenital amaurosis

Action: For discussion/adoption

Document(s) tabled:
Draft Summary report

Notes: There have been 3 designations for this condition: EMA/OD/182/13 Adeno-associated viral vector serotype 8 containing the human GUCY2D gene, EMA/OD/163/10 9-cis-Retinyl acetate, EMA/OD/150/11 Adenovirus associated viral vector serotype 2 containing the human RPE65 gene
Designations withdrawn: EMA/OD/063/11 Adeno-associated viral vector serotype 8 containing the human AIPL1 gene

2.2.7. - EMA/OD/157/17

Treatment of Pemphigus

Action: For discussion/adoption

Document(s) tabled:
Draft Summary report

Notes: There have been 2 designations for this condition: EMA/OD/116/11 Human monoclonal antibody against Fas ligand, EMA/OD/091/14 (S)-2-(1-((6-amino-5-cyanopyrimidin-4-yl)amino)ethyl)-4-oxo-3-phenyl-3,4-dihydropyrrolo[2,1-f][1,2,4]triazine-5-carbonitrile

2.2.8. - EMA/OD/144/17

Treatment of oculopharyngeal muscular dystrophy (OPMD)

Action: For discussion/adoption

Document(s) tabled:
Draft Summary report

2.2.9. - EMA/OD/095/17

Treatment of pancreatic cancer

Action: For discussion/adoption

Document(s) tabled:
Draft Summary report

Notes: There have been 35 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/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/071/13 Chimeric monoclonal antibody against claudin-18 splice variant 2, EMA/OD/164/13 Cysteamine bitartrate, EMA/OD/081/14 Immunoglobulin G1 (TEXT TOO LONG), EMA/OD/085/14 [5-Amino-1-(4-fluoro-phenyl)-1H-pyrazol-4-yl]-[3-(2,3-dihydroxy-propoxy)-phenyl]-methanone, EMA/OD/187/14 Herpes simplex type 1 virus containing cellular B-myb gene as tumour-specific promoter, EMA/OD/143/14 Heat-killed

Mycobacterium obuense (whole cell), EMA/OD/173/14 Pegylated recombinant human hyaluronidase PH20, EMA/OD/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/150/10 Salirasib, 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, EMA/OD/037/13 (1-methyl-2-nitro-1H-imidazole-5-yl)methyl N,N'-bis(2-bromoethyl)diamidophosphate

2.2.10. - EMA/OD/161/17

Treatment of haematopoietic stem cell transplantation

Action: For discussion/adoption

Document(s) tabled:
Draft Summary report

Notes: There have been 6 designations for this condition: EMA/OD/090/16 Radio-iodinated (131I) anti-CD45 murine monoclonal antibody, 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/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/149/16 Allogeneic peripheral blood mononuclear cells incubated ex vivo with 16, 16-dimethyl prostaglandin E2 and dexamethasone, 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

2.2.11. - EMA/OD/160/17

Treatment of cerebral autosomal-dominant arteriopathy with subcortical infarcts and leukoencephalopathy

Action: For discussion/adoption

Document(s) tabled:
Draft Summary report

2.2.12. - EMA/OD/152/17

Treatment of atypical haemolytic uremic syndrome

Action: For discussion/adoption

Document(s) tabled:
Draft Summary report

Notes: There has been 1 designation for this condition: EMEA/OD/027/09 Eculizumab

2.2.13. - EMA/OD/155/17

Treatment of Immunoglobulin G4-Related Disease

Action: For discussion/adoption

Document(s) tabled:
Draft Summary report

2.2.14. - EMA/OD/159/17

Treatment of haematopoietic stem cell transplantation

Action: For discussion/adoption

Document(s) tabled:
Draft Summary report

Notes: There have been 6 designations for this condition: Please see 2.2.10.

2.2.15. - EMA/OD/153/17

Treatment of argininosuccinic aciduria

Action: For discussion/adoption

Document(s) tabled:
Draft Summary report

Notes: There have been 5 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, EMA/OD/184/16 sodium benzoate

2.2.16. - EMA/OD/166/17

Treatment of small cell lung cancer

Action: For discussion/adoption

Document(s) tabled:
Draft Summary report

Notes: There have been 6 designations for this condition: EMA/OD/086/14 2-(2-methyl-5-nitro-1H-imidazol-1-yl)ethylsulfamide, EMA/OD/015/16 Rovalpituzumab tesirine,

EMA/OD/056/10 Maytansinoid-conjugated humanised monoclonal antibody against CD56, EMEA/OD/113/07 Amrubicin hydrochloride, EMEA/OD/055/07 Picoplatin, EMEA/OD/056/04 Sabarubicin

2.2.17. - EMA/OD/164/17

Treatment of congenital adrenal hyperplasia

Action: For discussion/adoption

Document(s) tabled:
Draft Summary report

Notes: There has been 1 designation for this condition: EMEA/OD/020/05 Hydrocortisone (modiefied release tablet)
Designation withdrawn: EMA/OD/063/15 Verucerfont

2.2.18. - EMA/OD/096/17

Treatment of adult-onset Still's disease

Action: For discussion/adoption

Document(s) tabled:
Draft Summary report

2.2.19. - EMA/OD/129/17

Treatment of systemic juvenile idiopathic arthritis

Action: For discussion/adoption

Document(s) tabled:
Draft Summary report

2.2.20. - EMA/OD/131/17

Treatment of mantle cell lymphoma

Action: For discussion/adoption

Document(s) tabled:
Draft Summary report

Notes: There have been 9 designations for this condition: EMEA/OD/058/06 Temsirolimus, EMEA/OD/053/03 Recombinant antibody derivative against human CD19 and CD3, EMEA/OD/064/04 Recombinant histidine-tagged idiotype immunoglobulin Fab fragment of clonal B-cell receptors, EMA/OD/059/10 (3S)-3-{4-[7-(aminocarbonyl)-2H-indazol-2-yl] phenyl} piperidine tosylate monohydrate salt, EMA/OD/113/10 Autologous tumour-derived immunoglobulin idiotype coupled to keyhole limpet haemocyanin, EMA/OD/078/11 Lenalidomide, EMA/OD/171/12 1-[(3R)-3-[4-amino-3-(4-phenoxyphenyl)-1H- pyrazolo [3,4-d]pyrimidin-1-yl]-1-piperidinyl]-2-propen-1-one, EMA/OD/077/15 Autologous T cells transduced with retroviral vector encoding an anti-CD19 CD28/CD3-zeta chimeric antigen receptor, EMA/OD/231/15 Acalabrutinib

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 30-31 October 2017 COMP meeting

2.7. Evaluation on-going

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

Action: For information

Notes: See 7.8.1. Table 6. Evaluation Ongoing.

3. Requests for protocol assistance with significant benefit question

3.1. Ongoing procedures

3.1.1. -

Treatment of spinal muscular atrophy

Action: For adoption

3.1.2. -

Treatment of plasma cell myeloma

Action: For adoption

3.1.3. -

Treatment of sickle cell disease

Action: For adoption

3.1.4. -

Treatment of chronic lymphocytic leukaemia

Action: For adoption

3.1.5. -

Treatment of idiopathic pulmonary fibrosis

Action: For adoption

3.1.6. -

Treatment of small cell lung cancer

Action: For adoption

3.1.7. -

Treatment of ornithine transcarbamylase deficiency

Action: For adoption

3.2. Finalised letters

None

3.3. New requests

3.3.1. -

Treatment of Lennox-Gastaut syndrome

Action: For information

3.3.2. -

Treatment of mantle cell lymphoma

Action: For information

3.3.3. -

Treatment of acute myeloid leukaemia

Action: For information

3.3.4. -

Treatment of myelodysplastic syndromes

Action: For information

3.3.5. -

Treatment of Leber's hereditary optic neuropathy

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

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

Action: For adoption, Oral explanation to be held on 30 October 2017 at time 14:30

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

4.2.2. - plitidepsin - EMEA/H/C/004354, EMEA/OD/044/04, EU/3/04/245

Pharma Mar SA; Treatment of multiple myeloma

Action: For discussion

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

4.2.3. - rucaparib - EMEA/H/C/004272, EMA/OD/085/12, EU/3/12/1049

Clovis Oncology UK Ltd; Treatment of ovarian cancer

Action: For discussion

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

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

Eisai Ltd; Treatment of hepatocellular carcinoma

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

Action: For discussion

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

4.2.5. - budesonide - EMEA/H/C/004655, EMA/OD/078/13, EU/3/13/1181

Dr. Falk Pharma GmbH; Treatment of eosinophilic esophagitis

Action: For discussion

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

4.2.6. - velmanase alfa - EMEA/H/C/003922, EMEA/OD/074/04, EU/3/04/260,

Chiesi Farmaceutici S.p.A.; Treatment of alpha-Mannosidosis

Action: For discussion

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

4.2.7. - glibenclamide - EMEA/H/C/004379, EMA/OD/149/15, EU/3/15/1589

Ammtek; Treatment of neonatal diabetes

Action: For discussion

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

4.2.8. - burosumab - EMEA/H/C/004275, EMEA/H/C/004275, EU/3/14/1351

Kyowa Kirin Limited; Treatment of X-linked hypophosphataemia

Action: For discussion

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

4.3. Appeal

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 adoption, Oral explanation to be held on 30 October 2017 at time 11:00

Document(s) tabled:
Revised draft Summary report
Sponsor's grounds for appeal

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

Pfizer Limited - UK; Treatment of chronic myeloid leukaemia

CHMP rapporteur: Harald Enzmann

Action: For discussion

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

5.2.2. Lynparza - (Olaparib) – EMEA/H/C/003726/X/0016/G, EMEA/OD/063/07, EU/3/07/501

AstraZeneca AB - Sweden; Treatment of ovarian cancer

CHMP rapporteur: Alexandre Moreau

Action: For information

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. Strategic Review & Learning meetings

None

7.1.2. Protocol Assistance Working Group (PAWG)

Proposed meeting time on 30 October 2017 at 13:00

Document(s) tabled:
PAWG draft agenda for 30 October 2017 meeting
PAWG draft minutes for 3 October 2017 meeting

7.1.3. Non-Clinical Working Group

None

7.1.4. Condition Working Group

Proposed meeting time on 27 October 2017 at 10:00

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 October 2017

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

Document(s) tabled:

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

Action: For information

Document(s) tabled:

7.3.3. Scientific Advice Working Party (SAWP)

Re-examination of SAWP composition and Committee representatives at SAWP

Action: For information

Document(s) tabled:
List of volunteers

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 discussion/adoption

Document(s) tabled:
COMP Work Plan 2018

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. Preparedness of the system and capacity increase

Action: For discussion

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

Action: For information

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/