13 April 2018
EMA/COMP/171165/2018
Inspections, Human Medicines Pharmacovigilance and Committees

# Committee for Orphan Medicinal Products (COMP)

## Draft agenda for the meeting on 17-19 April 2018

Chair: Bruno Sepodes – Vice-Chair: Lesley Greene

17 April 2018, 09:00-19:30, room 2F

18 April 2018, 08:30-19:30, room 2F

19 April 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).

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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 17-19 April 2018. See April 2018 COMP minutes (to be published post May 2018 COMP meeting).

## 1.2. Adoption of agenda

COMP agenda for 17-19 April 2018.

## 1.3. Adoption of the minutes

COMP minutes for 13-15 March 2018.

# 2. Applications for orphan medicinal product designation

## 2.1. For opinion

### 2.1.1. - EMA/OD/259/17

Treatment of acute myeloid leukaemia

**Action:** For adoption, Oral explanation to be held on 18 April 2018 at 14:30

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

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-3O, 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

### 2.1.2. - EMA/OD/252/17

Treatment of glioma

**Action:** For adoption, Oral explanation to be held on 17 April 2018 at 15:30

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

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

### 2.1.3. - EMA/OD/260/17

Treatment of follicular lymphoma

**Action:** For adoption, Oral explanation to be held on 17 April 2018 at 17:00

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

Notes: There have been 9 designations for this condition: EMEA/OD/040/06 Autologous tumor-derived immunoglobulin idiotype coupled to keyhole limpet haemocyanin, EMEA/OD/065/04 Recombinant hisitidine-tagged idiotype immunoglobulin Fab fragment of clonal B-cell receptors, EMA/OD/158/12 lenalidomide, EMA/OD/047/13 (S)-3-(1-(9H-purin-6-ylamino)ethyl)-8-chloro-2-phenylisoquinolin-1(2H)-one, EMA/OD/111/13 Ibrutinib, EMA/OD/200/13 177Lu-tetraxetan-tetulomab, EMA/OD/013/15 obinutuzumab, EMA/OD/135/15 Autologous T cells transduced with retroviral vector encoding an anti-CD19 CD28/CD3-zeta chimeric antigen receptor, EMA/OD/103/17 Glucopyranosyl lipid A
Designations withdrawn: EMEA/OD/061/02 Iodine (131I) tositumomab, EMEA/OD/079/02 Tositumomab, EMA/OD/053/13 Idelalisib

### 2.1.4. - EMA/OD/196/17

Treatment of biliary tract cancer

**Action:** For information

Document(s) tabled:
Withdrawal request of 3 April 2018

Notes: There have been 4 designations for this condition: EMA/OD/199/13 (5R,5aR,8aR,9S)-9-[[4,6-O-[(R)-Ethylidene]-Ω-D-glucopyranosyl]-oxy]-5-(4-({[(2,2-dimethyl-1,3-dioxolan-4-yl)methoxy]carbonyl}oxy)-3,5-dimethoxyphenyl)-5,8,8a,9-tetrahydroisobenzofuro[5,6-f][1,3]benzodioxol-6(5aH)-one, EMA/OD/305/14 5,10,15,20-tetrakis(2,6-difluoro-3-N-methylsulfamoylphenyl)bacteriochlorin, EMA/OD/245/15 (R)-6-(2-fluorophenyl)-N-(3-(2-((2-methoxyethyl)amino)ethyl)phenyl)-5,6-dihydrobenzo[h]quinazolin-2-amine dihydrochloride, EMA/OD/124/17 5-amino-1-(2-methyl-1H-benzo[d]imidazol-5-yl)-1H-pyrazol-4-yl 1H-indol-2-yl ketone mono[(S)-2-hydroxysuccinate]

### 2.1.5. - EMA/OD/250/17

Treatment of invasive aspergillosis

**Action:** For adoption, Oral explanation to be held on 18 April 2018 at 11:00

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

Notes: There have been 2 designations for this condition: EMA/OD/009/14 Isavuconazonium sulfate, EMA/OD/104/16 2-(1,5-dimethyl-3-phenyl-1H-pyrrol-2-yl)- N-{4-[4-(5-fluoro-pyrimidin-2-yl) piperazin- 1-yl]-phenyl}-2-oxo-acetamide

### 2.1.6. - EMA/OD/251/17

Prevention of invasive aspergillosis

**Action:** For adoption, Oral explanation to be held on 18 April 2018 at 11:00

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

Notes: There have been 2 designations for this condition: See 2.1.5.

### 2.1.7. - EMA/OD/223/17

Treatment of multiple myeloma

**Action:** For adoption, Oral explanation to be held on 18 April 2018 at 12:00

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

Notes: There have been 16 designations for this condition: EMEA/OD/040/01 Thalidomide, EMEA/OD/044/04 Aplidine, EMEA/OD/066/04 Recombinant histidine-tagged idiotype immunoglobulin Fab fragment of clonal B-cell receptors, EMEA/OD/012/05 N-(methyl-diazacyclohexyl-methylbenzamide)-azaphenyl-aminothiopyrrole, EMEA/OD/120/07 Carfilzomib, EMEA/OD/068/08 N2'-Deacetyl-N2'-[4-methyl-4-(oxobuthyldithio)-1-oxopentyl]-maytansine-chimerized anti-CD138 IgG4 monoclonal antibody, EMEA/OD/076/08 Human anti-intercellular adhesion molecule-1 monoclonal antibody, EMEA/OD/053/08 Milatuzumab, EMEA/OD/053/09 Pomalidomide, EMA/OD/017/11 Acadesine, EMA/OD/048/11 2,2'-{2-[(1R)-1-({[(2,5-dichlorobenzoyl)amino]acetyl}amino)-3-methylbutyl]-5-oxo-1,3,2-dioxaborolane-4,4-diyl}diacetic acid, EMA/OD/113/12 Panobinostat, EMA/OD/125/17 Autologous ex-vivo-expanded peripheral polyclonal

lymphocytes enriched in activated natural killer cells, EMA/OD/121/16 Venetoclax, EMA/OD/270/16 Autologous T lymphocyte-enriched population of cells transduced with a lentiviral vector encoding a chimeric antigen receptor targeting human B cell maturation antigen with 4-1BB and CD3-zeta intracellular signalling domains, EMA/OD/077/17 Humanised monoclonal antibody targeting B-cell maturation antigen conjugated with maleimidocaproyl monomethyl auristatin F
Designations withdrawn: EMEA/OD/048/00 Arsenic trioxide, EMEA/OD/003/01 Humanised anti-HM1.24 monoclonal antibody, EMEA/OD/018/00 Thalidomide, EMEA/OD/026/01 Deoxyribose phosphorothioate (5'-tct-ccc-agc-gtg-cgc-cat-3'), EMEA/OD/019/01 Thalidomide, EMEA/OD/070/04 17-allylamino-17-demethoxygeldanamycin, EMEA/OD/093/05 Human monoclonal antibody against HLA-DR, EMEA/OD/003/09 Chimeric-anti-interleukin-6 monoclonal antibody, EMEA/OD/133/09 Dexamethasone (40 mg tablet), EMEA/OD/130/09 Perifosine, EMA/OD/115/10 Maytansinoid-conjugated humanised monoclonal antibody against CD56, EMA/OD/137/10 Vorinostat, EMA/OD/137/11 Chimeric monoclonal antibody against kappa myeloma antigen, EMA/OD/061/12 Elotuzumab

### 2.1.8. - EMA/OD/256/17

Treatment of glioma

**Action:** For adoption, Oral explanation to be held on 17 April 2018 at 09:30

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

Notes: There have been 43 designations for this condition: See 2.1.2.

### 2.1.9. - EMA/OD/248/17

Treatment of Shiga-Toxin Producing Escherichia Coli Haemolytic Uremic Syndrome

**Action:** For adoption, Oral explanation to be held on 18 April 2018 at 15:30

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

## 2.2. For discussion / preparation for an opinion

### 2.2.1. - EMA/OD/003/18

Treatment of hereditary angioedema

**Action:** For discussion/adoption

Document(s) tabled:
Draft Summary report

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

## 2.2.2. - EMA/OD/018/18

Treatment of Stargardt's disease

**Action:** For discussion/adoption

Document(s) tabled:
Draft Summary report

Notes: There have been 6 designations for this condition: EMEA/OD/127/09 Lentiviral vector containing the human ABCA4 gene, EMA/OD/173/10 Human embryonic stem-cell-derived retinal pigment epithelial cells, EMA/OD/175/12 Ramiprilat, EMA/OD/124/13 Soraprazan, EMA/OD/005/14 Mixture of two adeno-associated viral vectors of serotype 8 containing the 5'-half sequence of human ABCA4 gene and the 3'-half sequence of human ABCA4 gene, EMA/OD/295/14 Ecothiopate iodide
Designation withdrawn: EMEA/OD/084/08 Adeno-associated viral vector serotype 5 containing the human ABCA4 gene

## 2.2.3. - EMA/OD/020/18

Treatment of Duchenne muscular dystrophy

**Action:** For discussion/adoption

Document(s) tabled:
Draft Summary report

Notes: There have been 27 designations for this condition: 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

### 2.2.4. - EMA/OD/008/18

Treatment of Ebola virus disease

**Action:** For discussion/adoption

Document(s) tabled:
Draft Summary report

Notes: There have been 3 designations for this condition: EMA/OD/250/14 Fibrinogen-coated albumin spheres, EMA/OD/310/14 Rintatolimod, EMA/OD/197/15 2-ethylbutyl (2S)-2-{[(S)-{[(2R,3S,4R,5R)-5-(4-aminopyrrolo[2,1-f][1,2,4]triazin-7-yl)-5-cyano-3,4-dihydroxytetrahydrofuran-2-yl]methoxy}(phenoxy)phosphoryl]amino}propanoate

### 2.2.5. - EMA/OD/010/18

Treatment of haemophilia A

**Action:** For discussion/adoption

Document(s) tabled:
Draft Summary report

Notes: There have been 12 designations for this condition: EMA/OD/128/10 Pegylated B-domain-deleted sequence-modified recombinant human factor VIII, EMA/OD/132/10 Recombinant fusion protein linking human coagulation factor VIIa with human albumin (rVIIa-FP), EMA/OD/144/11 Pegylated recombinant factor VIII, EMA/OD/095/12 Humanised monoclonal IgG4 antibody against tissue factor pathway inhibitor, EMA/OD/039/14 Synthetic double-stranded siRNA oligonucleotide directed against antithrombin mRNA that is covalently linked to a ligand containing three N-acetylgalactosamine residues, EMA/OD/069/14 Recombinant factor VIIa modified with three terminal repeats derived from the β chain of human chorionic gonadotropin, EMA/OD/123/14 A combination of H-Lys-Lys-Gly-Pro-Arg-Cys(SH)-Leu-Thr-Arg-Tyr-Tyr-Ser-Ser-Phe-Val-Asn-Met-Glu-Gly-Lys-Lys-OH and H-Lys-Lys-Gly-Asp-Asn-Ile-Met-Val-Thr-Phe-Arg-Asn-Gln-Ala-Ser-Arg-Pro-Tyr-Gly-Lys-Lys-OH, EMA/OD/238/16 Autologous dendritic cells incubated ex vivo with zebularine and factor VIII, EMA/OD/230/15 adeno-associated viral vector serotype 5 containing a B-domain deleted variant of human coagulation factor VIII gene, EMA/OD/093/16 Human monoclonal IgG1 antibody against tissue factor pathway inhibitor, EMA/OD/019/17 Recombinant adeno-

associated viral vector serotype 6 encoding the B-domain-deleted human factor VIII, EMA/OD/189/17 Human monoclonal IgG2 antibody against tissue factor pathway inhibitor Designations withdrawn: EMEA/OD/031/09 Sequence-modified recombinant human factor VIIa, EMA/OD/030/10 Recombinant fusion protein consisting of human coagulation factor VIII attached to the Fc domain of human IgG1, EMA/OD/043/10 Recombinant porcine factor VIII (B domain deleted), EMA/OD/069/12 vatreptacog alfa (activated), EMA/OD/144/13 Humanised monoclonal modified IgG4 antibody with bispecific structure targeting factors IX, IXa, X and Xa

### 2.2.6. - EMA/OD/013/18

Treatment of neuronal ceroid lipofuscinosis

**Action:** For discussion/adoption

Document(s) tabled:
Draft Summary report

Notes: There has been 1 designation for this condition: EMA/OD/195/16 Recombinant self-complementary adeno-associated viral vector serotype 9 encoding the human CLN3gene

### 2.2.7. - EMA/OD/236/17

Treatment of amyotrophic lateral sclerosis

**Action:** For discussion/adoption

Document(s) tabled:
Draft Summary report

Notes: There have been 23 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, EMA/OD/136/17 (R)-troloxamide quinone, EMA/OD/174/17 Levosimendan
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

## 2.2.8. - EMA/OD/011/18

Treatment of Alport syndrome

**Action:** For discussion/adoption

Document(s) tabled:
Draft Summary report

Notes: There has been 1 designation for this condition: EMA/OD/238/14 5'-ASCSASTSCSASGSTSCSTSGSASUSASASGSCSTSA-3'

## 2.2.9. - EMA/OD/014/18

Treatment of epidermolysis bullosa

**Action:** For discussion/adoption

Document(s) tabled:
Draft Summary report

Notes: There have been 14 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 Designation withdrawn: EMA/OD/172/10 Human dermal fibroblasts cultured on a bioresorbable polyglactin mesh

## 2.2.10. - EMA/OD/007/18

Treatment of 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/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

### 2.2.11. - EMA/OD/207/17

Treatment of immunoglobulin light chain amyloidosis

**Action:** For discussion/adoption

Document(s) tabled:
Draft Summary report

Notes: There have been 2 designations for this condition: EMA/OD/020/14 Carboxy pyrrolidine hexanoyl pyrrolidine carboxylate, EMA/OD/021/14 Recombinant monoclonal antibody to human serum amyloid P component

### 2.2.12. - EMA/OD/006/18

Treatment of neurodegeneration with brain iron accumulation

**Action:** For discussion/adoption

Document(s) tabled:
Draft Summary report

### 2.2.13. - EMA/OD/001/18

Treatment of transthyretin-mediated amyloidosis

**Action:** For discussion/adoption

Document(s) tabled:
Draft Summary report

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

### 2.2.14. - EMA/OD/009/18

Treatment of growth hormone deficiency

**Action:** For discussion/adoption

Document(s) tabled:
Draft Summary report

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

## 2.2.15. - EMA/OD/015/18

Treatment of malignant cerebral oedema

**Action:** For discussion/adoption

Document(s) tabled:
Draft Summary report

## 2.2.16. - EMA/OD/257/17

Treatment of congenital hyperinsulinism

**Action:** For discussion/adoption

Document(s) tabled:
Draft Summary report

Notes: There have been 4 designations for this condition: EMA/OD/140/11 Glucagon, EMA/OD/128/14 Glucagon, EMA/OD/040/16 Recombinant human monoclonal antibody to insulin receptor, EMA/OD/002/17 Synthetic glucagon analogue modified to contain 7 amino acid substitutions

## 2.2.17. - EMA/OD/012/18

Treatment of cystinuria

**Action:** For discussion/adoption

Document(s) tabled:
Draft Summary report

## 2.2.18. - EMA/OD/002/18

Treatment of transthyretin-mediated amyloidosis

**Action:** For discussion/adoption

Document(s) tabled:
Draft Summary report

Notes: There have been 3 designations for this condition: See 2.2.13.

## 2.2.19. - EMA/OD/017/18

Treatment of methylmalonic acidemia

**Action:** For discussion/adoption

Document(s) tabled:
Draft Summary report

Notes: There has been 1 designation for this condition: EMEA/OD/045/08 Carglumic acid

### 2.2.20. - EMA/OD/005/18

Treatment of progressive supranuclear palsy

**Action:** For discussion/adoption

Document(s) tabled:
Draft Summary report

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

### 2.2.21. - EMA/OD/019/18

Treatment of transthyretin-mediated amyloidosis (ATTR amyloidosis)

**Action:** For discussion/adoption

Document(s) tabled:
Draft Summary report

Notes: There have been 3 designations for this condition: See 2.2.13.

## 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 17-19 April 2018 COMP meeting

## 2.7. Evaluation on-going

Sixteen 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 amyotrophic lateral sclerosis

**Action**: For adoption

### 3.1.2. -

Treatment of sickle cell disease

**Action**: For adoption

### 3.1.3. -

Treatment of soft tissue sarcoma

**Action**: For adoption

### 3.1.4. -

Treatment of acute myeloid leukaemia

**Action**: For adoption

### 3.1.5. -

Treatment of pemphigus

**Action**: For adoption

### 3.1.6. -

Treatment of small cell lung cancer

**Action**: For adoption

### 3.1.7. -

Treatment of tuberous sclerosis

**Action**: For adoption

### 3.1.8. -

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

**Action**: For adoption

### 3.1.9. -

Treatment of Cushing's syndrome

**Action**: For adoption

### 3.1.10. -

Treatment of acute myeloid leukaemia

**Action**: For adoption

## 3.2. Finalised letters

### 3.2.1. -

Treatment of acute hepatic porphyria

**Action**: For information

### 3.2.2. -

Treatment of eosinophilic oesophagitis

**Action**: For information

### 3.2.3. -

Treatment of gastrointestinal stromal tumours

**Action**: For information

### 3.2.4. -

Treatment of pulmonary arterial hypertension

**Action**: For information

### 3.2.5. -

Treatment of partial deep dermal and full thickness burns

**Action**: For information

## 3.3. New requests

### 3.3.1. -

Treatment of Fabry disease

**Action**: For information

### 3.3.2. -

Treatment of acute myeloid leukaemia

**Action**: For information

### 3.3.3. -

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

**Action**: For information

### 3.3.4. -

Treatment of congenital hyperinsulinism

**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. - 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

### 4.2.2. –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 discussion

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

### 4.2.3. - vestronidase alfa – EMA/OD/127/11, EU/3/12/973, EMEA/H/C/004438

Ultragenyx Germany GmbH; Treatment of mucopolysaccharidosis type VII (Sly syndrome)

**Action**: For discussion

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

### 4.2.4. – eteplirsen – EMEA/OD/049/08, EU/3/08/586, EMEA/H/C/004355

AVI Biopharma International Ltd; Treatment of Duchenne muscular dystrophy

**Action**: For discussion

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

### 4.2.5. - caplacizumab - EMEA/OD/109/08, EU/3/09/629, EMEA/H/C/004426

Ablynx NV; Treatment of thrombotic thrombocytopenic purpura

**Action**: For discussion

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

### 4.2.6. - autologous t cells transduced with lentiviral vector containing a chimeric antigen receptor directed against cd19 – EMEA/H/C/004090

Novartis Europharm Limited;

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

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. 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. 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. Kalydeco - Ivacaftor – Type II variation – EMEA/H/C/002494/II/0069, EMEA/OD/010/08, EU/3/08/556

Vertex Pharmaceuticals (Europe) Ltd.; Treatment of cystic fibrosis

CHMP rapporteur: Concepcion Prieto Yerro

**Action**: For discussion

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

### 5.2.2. Venclyxto – Venetoclax – Type II variation – EMEA/H/C/004106/II/0008, EMA/OD/124/12, EU/3/12/1080

AbbVie Limited; Treatment of chronic lymphocytic leukaemia

CHMP rapporteur: Filip Josephson

**Action**: For discussion

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

### 5.2.3. 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

**Action**: For discussion

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

### 5.2.4. Mozobil - Plerixafor – Type II variation – EMEA/H/C/001030/II/0034, EMEA/OD/045/04, EU/3/04/227

Genzyme Europe BV; Treatment to mobilize progenitor cells prior to stem cell transplantation

CHMP rapporteur: Paula Boudewina van Hennik

**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 information

Document(s) tabled:
Agenda COMP Strategic Review and Learning Meeting 26-28 March 2018

### 7.1.2. Protocol Assistance Working Group (PAWG)

Proposed meeting time on 17 April 2018 2018 at 13:00

Document(s) tabled:
PAWG draft agenda for 17 April 2018 meeting
PAWG draft minutes for 13 March 2018 meeting

### 7.1.3. Condition Working Group

Proposed meeting time on 19 April 2018 at 08:30

### 7.1.4. Prevalence Working Group

Proposed meeting time on 18 April 2018 at 08:30

### 7.1.5. Committee for Orphan Medicinal Products Rules of Procedure

**Action**: For adoption

Document(s) tabled:
COMP Rules of Procedure Rev. 4

## 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 tabled:
PRIME eligibility requests - list of adopted outcomes March 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

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

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

## 8.1. Update on the EMA relocation

**Action**: For information

## 8.2. Preparedness of the system and capacity increase

**Action**: For discussion

## 8.3. EMA Business Pipeline activity and Horizon scanning

**Action:** For information

Document tabled:
Q1/2018 Update of the Business Pipeline report for the human scientific committees

## 8.4. Judgment of the General Court in Shire v EMA, T-80/16

**Action:** For information

Link to judgment: *Shire v EMA*, T-80/16, EU:T:2018:165

# 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/