



EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

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Inspections, Human Medicines Pharmacovigilance and Committees Division

Paediatric Committee (PDCO)

Agenda for the meeting on 12-15 September 2017

Chair: Dirk Mentzer – Vice-Chair: Koenraad Norga

12 September 2017, 13:00 - 19:00, room 3A

13 September 2017, 08:30 - 19:00, room 3A

14 September 2017, 08:30 - 19:00, room 3A

15 September 2017, 08:30 - 13:00, room 2A

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 PDCO Committee meeting reports (after the PDCO Opinion is adopted), and on the Opinions and decisions on paediatric investigation plans webpage (after the EMA Decision is issued).

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. Introductions

1.1. Welcome and declarations of interest of members, alternates and experts

Pre-meeting list of participants and restrictions in relation to declarations of interests applicable to the items of the agenda for the PDCO plenary session to be held 12-15 September 2017. See September 2017 PDCO minutes (to be published post October 2017 PDCO meeting).

1.2. Adoption of agenda

PDCO agenda for 12-15 September 2017.

1.3. Adoption of the minutes

PDCO minutes for 15-18 August 2017.

2. Opinions

Disclosure of some information related to this section cannot be released at the present time as it is deemed to contain commercially confidential information.

2.1. Opinions on Products

2.1.1. Ibrutinib - Orphan - EMEA-001397-PIP05-17

Janssen-Cilag International N.V.; Treatment of mantle cell lymphoma (MCL)

Day 1 opinion

Action: For adoption

Oncology

2.1.2. Ibrutinib - Orphan - EMEA-001397-PIP06-17

Janssen-Cilag International N.V.; Lymphoplasmacytic lymphoma / Treatment of Waldenström's macroglobulinaemia (WM)

Day 1 opinion

Action: For adoption

Other

2.1.3. Crisaborole - EMEA-002065-PIP01-16

Mild to moderate atopic dermatitis

Day 120 opinion

Action: For adoption

Dermatology

2.1.4. Ligelizumab - EMEA-001811-PIP02-15

Treatment of chronic spontaneous urticaria

Day 120 opinion

Action: For adoption

Dermatology

2.1.5. (2S)-2-{{(2R)-2-[[[3,3-dibutyl-7-(methylthio)-1,1-dioxido-5-phenyl-2,3,4,5-tetrahydro-1,2,5-benzothiadiazepin-8-yl]oxy}acetyl)amino]-2-(4-hydroxyphenyl)acetyl]amino}butanoic acid - Orphan - EMEA-002054-PIP01-16

Albireo AB; Treatment of Progressive Familial Intrahepatic Cholestasis

Day 120 opinion

Action: For adoption

Gastroenterology-Hepatology

2.1.6. maribavir - Orphan - EMEA-000353-PIP02-16

Shire Pharmaceuticals Ireland Limited; Treatment of CMV infection / Treatment of CMV infection in transplant patients from birth to <18 years of age

Day 120 opinion

Action: For adoption

Infectious Diseases

2.1.7. allopregnanolone - EMEA-002051-PIP01-16

Treatment of Status Epilepticus

Day 120 opinion

Action: For adoption

Neurology

2.1.8. acalabrutinib - Orphan - EMEA-001796-PIP03-16

ACERTA PHARMA, BV; Treatment of mature B cell neoplasms / Treatment of children from 1 to <18 years of age with previously untreated mature B-cell neoplasms (eg, diffuse large B-cell lymphoma [DLBCL], Burkitt lymphoma [BL] and primary mediastinal B-cell lymphoma [PMBCL])., Treatment of children from 1 to <18 years of age with relapsed/refractory mature B-cell neoplasms (eg, diffuse large B-cell lymphoma [DLBCL], Burkitt lymphoma [BL] and primary mediastinal B-cell lymphoma [PMBCL])

Day 120 opinion

Action: For adoption

Oncology

2.1.9. Lactobacillus reuteri - Orphan - EMEA-001895-PIP01-15

Infant Bacterial Therapeutics AB; Prevention of necrotising enterocolitis

Day 120 opinion

Action: For adoption

Other / Neonatology - Paediatric Intensive Care / Gastroenterology-Hepatology

2.1.10. Hydrochlorothiazide / Irbesartan / Amlodipine - EMEA-002167-PIP01-17

Essential hypertension / Treatment of essential hypertension as substitution therapy in adult patients whose blood pressure is adequately controlled on the combination of amlodipine, irbesartan and hydrochlorothiazide, taken either as three single -component formulations or as a dual-component and a single component formulation

Day 60 opinion

Action: For adoption

Cardiovascular Diseases

2.1.11. Meldonium dihydrate - EMEA-002212-PIP01-17

an adjuvant treatment of stable effort angina pectoris

Day 60 opinion

Action: For adoption

Cardiovascular Diseases

2.1.12. Ezetimibe / Rosuvastatin - EMEA-002202-PIP01-17

E78.0 Pure hypercholesterolaemia, I25 Chronic ischaemic heart disease, E78.2 Mixed hyperlipidaemia

Day 60 opinion

Action: For adoption

Endocrinology-Gynaecology-Fertility-Metabolism / Cardiovascular Diseases

2.1.13. [capmatinib - EMEA-002203-PIP01-17](#)

Treatment of lung malignant neoplasms

Day 60 opinion

Action: For adoption

Oncology

2.1.14. [veliparib - Orphan - EMEA-000499-PIP03-17](#)

AbbVie Ltd; Treatment of breast cancer

Day 60 opinion

Action: For adoption

Oncology

2.1.15. [Oxymetazoline HCL / Pilocarpine HCL - EMEA-002181-PIP01-17](#)

Presbyopia

Day 60 opinion

Action: For adoption

Ophthalmology

2.1.16. [ezetimibe / bempedoic acid - EMEA-002200-PIP01-17](#)

Treatment of elevated cholesterol

Day 60 opinion

Action: For adoption

Other / Cardiovascular Diseases

2.2. **Opinions on Compliance Check**

The following compliance checks have been put up for discussion and the members of the PDCO have been invited to comment on issues of possible non-compliance

2.2.1. [fidaxomicin - EMEA-C1-000636-PIP01-09-M05](#)

Astellas Pharma Europe B.V.; Treatment of enterocolitis caused by clostridium difficile

Day 60 letter

Action: For adoption

Infectious Diseases

2.3. Opinions on Modification of an Agreed Paediatric Investigation Plan

2.3.1. [dabigatran etexilate mesilate - EMEA-000081-PIP01-07-M10](#)

Boehringer Ingelheim International GmbH; Treatment of thromboembolic events, Prevention of thromboembolic events / Treatment of venous thromboembolic events in paediatric patients (secondary venous thrombotic event prevention)

Day 60 opinion

Action: For adoption

Cardiovascular Diseases / Haematology-Hemostaseology

2.3.2. [edoxaban \(tosylate\) - EMEA-000788-PIP02-11-M06](#)

Daiichi Sankyo Europe GmbH; Prevention of arterial thromboembolism, Prevention of venous thromboembolism, Treatment of venous thromboembolism / Prevention of arterial thromboembolism in paediatric cardiac patients at risk of thrombotic events, Acute treatment & secondary prevention of symptomatic recurrent venous thrombotic events (VTE) in paediatric patients at risk

Day 60 opinion

Action: For adoption

Cardiovascular Diseases / Haematology-Hemostaseology

2.3.3. [Dapagliflozin - EMEA-000694-PIP02-14-M02](#)

AstraZeneca AB; Type 1 Diabetes Mellitus / As an adjunct to insulin treatment to improve glycaemic control in adults with type 1 diabetes mellitus when insulin alone does not provide adequate control

Day 60 opinion

Action: For adoption

Endocrinology-Gynaecology-Fertility-Metabolism

2.3.4. [recombinant parathyroid hormone \[rhPTH\(1-84\)\] - Orphan - EMEA-001526-PIP01-13-M02](#)

Shire Pharmaceuticals Ireland Limited; ICD10: E 89.2 hypoparathyroidism, post-procedural; E 20.9, unspecified

Day 60 opinion

Action: For adoption

2.3.5. Methoxy polyethylene glycol- epoetin beta - EMEA-000172-PIP01-07-M03

Roche Registration Limited; Anaemia associated with chronic kidney disease

Day 60 opinion

Action: For adoption

Haematology-Hemostaseology

2.3.6. Recombinant Human A Disintegrin and Metalloprotease with Thrombospondin Type-1 Motifs 13 - Orphan - EMEA-001160-PIP01-11-M01

Baxalta Innovations GmbH; Treatment of thrombotic thrombocytopenic purpura (TTP)

Day 60 opinion

Action: For adoption

Haematology-Hemostaseology

2.3.7. Autologous CD34+ haematopoietic stem cells transduced ex vivo with EFS lentiviral vector encoding for the human ADA gene - Orphan - EMEA-001974-PIP01-16-M01

Pr Bobby Gaspar; Treatment of severe combined immunodeficiency due to adenosine deaminase deficiency (ADA-SCID)

Day 60 opinion

Action: For adoption

Immunology-Rheumatology-Transplantation

2.3.8. Eculizumab - Orphan - EMEA-000876-PIP05-15-M02

Alexion Europe SAS; Myasthenia Gravis / Treatment of Refractory Generalized Myasthenia Gravis

Day 60 opinion

Action: For adoption

Immunology-Rheumatology-Transplantation

2.3.9. guselkumab - EMEA-001523-PIP02-14-M01

Janssen Cilag International NV; Treatment of psoriasis / Treatment of severe plaque psoriasis in children ≥ 6 to < 18 years of age who cannot be adequately controlled with topical agents and/or phototherapy

Day 60 opinion

Action: For adoption

Immunology-Rheumatology-Transplantation

2.3.10. [Tofacitinib - EMEA-000576-PIP01-09-M07](#)

Pfizer Limited; Treatment of chronic idiopathic arthritis (including rheumatoid arthritis, psoriatic arthritis, ankylosing spondylitis and juvenile idiopathic arthritis)

Day 60 opinion

Action: For adoption

Immunology-Rheumatology-Transplantation

2.3.11. [avibactam / ceftazidime - EMEA-001313-PIP01-12-M06](#)

Pfizer Limited; Treatment of bacterial infections / For the treatment of complicated urinary tract infections, For the treatment hospital acquired pneumonia, For the treatment of complicated intra-abdominal infections, For the treatment of Gram-negative bacterial infections

Day 60 opinion

Action: For adoption

Infectious Diseases

2.3.12. [Cabotegravir - EMEA-001418-PIP01-13-M01](#)

ViiV Healthcare UK Limited; Treatment of human immunodeficiency virus (HIV-1) Infection / Treatment of human immunodeficiency virus (HIV-1) Infection, in combination with other antiretroviral agents

Day 60 opinion

Action: For adoption

Infectious Diseases

2.3.13. [Fostemsavir \(tromethamine\) - EMEA-001687-PIP01-14-M02](#)

ViiV Healthcare UK Ltd; Treatment of human immunodeficiency virus (HIV-1) infection / Treatment of HIV-1 infection as part of a combination therapy in paediatric patients who have no more than 2 remaining available fully active antiretroviral therapies.

Day 60 opinion

Action: For adoption

Infectious Diseases

2.3.14. Domagrozumab - Orphan - EMEA-001763-PIP01-15-M01

Pfizer Limited; Duchenne Muscular Dystrophy

Day 60 opinion

Action: For adoption

Neurology

2.3.15. Erenumab - EMEA-001664-PIP02-15-M01

Novartis Europharm Limited; Prevention of migraine headaches

Day 60 opinion

Action: For adoption

Neurology

2.3.16. Fremanezumab - EMEA-001877-PIP01-15-M01

Teva GmbH; Prevention of migraine headaches

Day 60 opinion

Action: For adoption

Neurology

2.3.17. Gemtuzumab Ozogamicin - Orphan - EMEA-001733-PIP02-15-M01

Pfizer Limited; Treatment of Acute Myloid Leukaemia

Day 60 opinion

Action: For adoption

Oncology

2.3.18. andexanet alfa - EMEA-001902-PIP01-15-M01

Portola Pharma UK Limited; prevention of factor Xa inhibitor associated haemorrhage, treatment of factor Xa inhibitor associated haemorrhage / for the reversal of anticoagulation due to direct and indirect factor Xa inhibitors in patients experiencing an acute major bleeding episode, for the reversal of anticoagulation due to direct and indirect factor Xa inhibitors in patients requiring urgent surgery

Day 60 opinion

Action: For adoption

Other

2.3.19. Febuxostat - EMEA-001417-PIP01-12-M03

Menarini International Operations Luxembourg S.A.; Prevention/treatment of hyperuricemia / Prevention or treatment of hyperuricemia in patients at intermediate or high risk of Tumor Lysis Syndrome (TLS) affected by hematologic malignancies

Day 60 opinion

Action: For adoption

Other / Oncology

2.3.20. Tapentadol - EMEA-000325-PIP01-08-M08

Grünenthal GmbH; Treatment of chronic pain

Day 60 opinion

Action: For adoption

Pain

2.3.21. Formoterol fumarate dihydrate / Beclometasone dipropionate - EMEA-000548-PIP01-09-M07

Chiesi Farmaceutici S.p.A.; COPD, Asthma / Maintenance therapy of asthma where use of a combination product (inhaled corticosteroid and long-acting beta2-agonist) is appropriate: - patients not adequately controlled with inhaled corticosteroids and 'as needed' inhaled short-acting beta2-agonist or - patients already adequately controlled on both inhaled corticosteroids and long-acting beta2-agonists.

Day 60 opinion

Action: For adoption

Pneumology - Allergology

2.3.22. Ivacaftor - Orphan - EMEA-001640-PIP01-14-M03

Vertex Pharmaceuticals (Europe) Ltd.; Treatment of Cystic Fibrosis

Day 60 opinion

Action: For adoption

Pneumology - Allergology

2.3.23. tralokinumab - EMEA-000782-PIP01-09-M04

MedImmune Ltd; Asthma / Reduce exacerbations and to improve asthma control and lung function in patients 12 years and older with severe asthma inadequately controlled with medium or high dose inhaled corticosteroids and another controller

Day 60 opinion

Action: For adoption

Pneumology - Allergology

2.3.24. Everolimus - Orphan - EMEA-000019-PIP08-12-M03

Novartis Europharm Limited; Tuberous Sclerosis Complex (TSC) / Treatment of refractory partial-onset seizures associated with tuberous sclerosis complex (TSC)

Day 60 opinion

Action: For adoption

Uro-nephrology / Neurology

2.3.25. Influenza virus surface antigens (haemagglutinin and neuraminidase) of the following strains: A/<Official Strain>(H1N1), A/<Official Strain>(H3N2), B/<Official Strain>Yamagata lineage, B/<Official Strain>Victoria lineage - EMEA-001782-PIP01-15-M01

Abbott Biologicals B.V.; Prevention of Influenza infection / Prophylaxis of influenza; especially in those who run an increased risk of associated complications

Day 60 opinion

Action: For adoption

Vaccines

2.4. Opinions on Re-examinations

2.4.1. Autologous T cells transduced with lentiviral vector containing a chimeric antigen receptor directed against CD19 - Orphan - EMEA-001654-PIP01-14-M02

Novartis Europharm Limited; B cell acute lymphoblastic leukaemia (ALL) / Treatment of CD19+ B cell acute lymphoblastic leukaemia (ALL) in paediatric patients whose disease is refractory to a standard chemotherapy regimen, relapsed after stem cell transplantation (SCT) or are ineligible for allogenic SCT

Day 30 opinion

Action: For adoption

Oncology

2.5. Finalisation and adoption of opinions

3. Discussion of applications

Disclosure of some information related to this section cannot be released at the present time as it is deemed to contain commercially confidential information.

3.1. Discussions on Products D90-D60-D30

3.1.1. Omega-3-carboxylic acids - EMEA-001865-PIP02-16

Hypertriglyceridaemia or mixed dyslipidaemia to reduce the risk of atherosclerotic cardiovascular disease (ACVD), Mixed dyslipidaemia with persistent hypertriglyceridaemia

Day 90 discussion

Action: For discussion

Cardiovascular Diseases

3.1.2. Synthetic double-stranded siRNA oligonucleotide directed against hydroxyacid oxidase 1 mRNA that is covalently linked to a ligand containing three N-acetylgalactosamine residues - Orphan - EMEA-002079-PIP01-16

Alnylam UK Limited; Treatment of Primary Hyperoxaluria Type 1

Day 90 discussion

Action: For discussion

Gastroenterology-Hepatology

3.1.3. Iron hydroxyethyl amylopectin heptonate - EMEA-002094-PIP01-16

Iron deficiency anemia., Iron deficiency.

Day 90 discussion

Action: For discussion

Haematology-Hemostaseology

3.1.4. emapalumab - Orphan - EMEA-002031-PIP01-16

Novimmune B.V; Treatment of Haemophagocytic Lymphohistiocytosis

Day 90 discussion

Action: For discussion

Immunology-Rheumatology-Transplantation

3.1.5. recombinant humanised IgG4 monoclonal antibody against MSRV-Envelope protein - EMEA-002127-PIP01-17

Treatment of Multiple Sclerosis / Treatment of patients from 10 to less than 18 years old with relapsing-remitting multiple sclerosis

Day 90 discussion

Action: For discussion

Neurology

3.1.6. ruxolitinib phosphate - EMEA-000901-PIP03-16

Acute graft versus host disease / Treatment of acute Graft vs Host Disease (GvHD) after allogeneic hematopoietic stem cell transplantation (alloSCT) in paediatric patients aged 28 days and above.

Day 90 discussion

Action: For discussion

Oncology

3.1.7. allopregnanolone - EMEA-002051-PIP02-16

Treatment of postpartum depression

Day 90 discussion

Action: For discussion

Psychiatry

3.1.8. calcifediol - EMEA-002093-PIP01-16

secondary Hyperparathyroidism (SHPT)

Day 90 discussion

Action: For discussion

Uro-nephrology

3.1.9. N. meningitidis serogroup W polysaccharide conjugated to tetanus toxoid / N. meningitidis serogroup Y polysaccharide conjugated to tetanus toxoid / N. meningitidis serogroup C polysaccharide conjugated to tetanus toxoid / N. meningitidis serogroup A polysaccharide conjugated to tetanus toxoid - EMEA-001930-PIP01-16

Prevention of Meningococcal Disease

Day 90 discussion

Action: For discussion

Vaccines

3.1.10. Bimekizumab - EMEA-002189-PIP01-17

Treatment of psoriasis / Treatment of moderate to severe chronic plaque psoriasis in children from the age of 6 years and older

Day 60 discussion

Action: For discussion

Dermatology

3.1.11. [Efpegsoyatropin - EMEA-001227-PIP02-17](#)

Treatment of growth hormone deficiency

Day 60 discussion

Action: For discussion

Endocrinology-Gynaecology-Fertility-Metabolism

3.1.12. [Alicaforsen - Orphan - EMEA-002060-PIP02-17](#)

Atlantic Pharmaceuticals (Holdings) Ltd; Treatment of gastrointestinal procedural complications / Treatment of active episodes of antibiotic refractory pouchitis

Day 60 discussion

Action: For discussion

Gastroenterology-Hepatology

3.1.13. [Obeticholic Acid - EMEA-001304-PIP03-17](#)

NASH / NASH with Fibrosis

Day 60 discussion

Action: For discussion

Gastroenterology-Hepatology

3.1.14. [Plazomicin Sulfate - EMEA-001639-PIP02-17](#)

Treatment of infections due to Enterobacteriaceae / Treatment of neonatal sepsis due to Enterobacteriaceae in patients with limited treatment options, Treatment of complicated urinary tract infections, including acute pyelonephritis, due to Enterobacteriaceae, including cases with concurrent bacteraemia.

Day 60 discussion

Action: For discussion

Infectious Diseases

3.1.15. [Pritelivir - EMEA-002180-PIP01-17](#)

Treatment of recurrent herpes labialis / Topical treatment of recurrent herpes labialis

Day 60 discussion

Action: For discussion

Infectious Diseases

3.1.16. Mecasermin rinfabate - Orphan - EMEA-000534-PIP03-17

Premature AB; Chronic lung disease of prematurity

Day 60 discussion

Action: For discussion

Neonatology - Paediatric Intensive Care

3.1.17. Lasmiditan - EMEA-002166-PIP01-17

Migraine with and without aura

Day 60 discussion

Action: For discussion

Neurology

3.1.18. taselisib - EMEA-002210-PIP01-17

Treatment of all conditions included in the category of malignant neoplasms (except central nervous system tumours, haematopoietic and lymphoid tissue neoplasms) / Treatment of children with relapsed or refractory solid tumors with known or anticipated PI3K activation

Day 60 discussion

Action: For discussion

Oncology

3.1.19. Tanezumab - EMEA-001635-PIP03-17

Treatment of chronic pain

Day 60 discussion

Action: For discussion

Pain

3.1.20. Cow's milk protein extract - EMEA-002201-PIP01-17

Treatment of IgE-mediated cow's milk allergy

Day 60 discussion

Action: For discussion

Pneumology - Allergology

3.1.21. ezetimibe / atorvastatin - EMEA-002207-PIP01-17

treatment of hypercholesterolaemia

Day 30 discussion

Action: For discussion

Cardiovascular Diseases

3.1.22. vonapanitase - Orphan - EMEA-002195-PIP01-17

Proteon Therapeutics Limited; prevention of arteriovenous fistula failure

Day 30 discussion

Action: For discussion

Cardiovascular Diseases

3.1.23. EMEA-002216-PIP01-17

Treatment of Atopic Dermatitis

Day 30 discussion

Action: For discussion

Dermatology

3.1.24. EMEA-002208-PIP01-17

Treatment of psoriasis, Treatment of Crohn's disease, Treatment of ulcerative colitis / Treatment of moderate to severely active Crohn's disease in paediatric patients aged 2 to less than 18 years of age, Treatment of moderate to severely active ulcerative colitis in paediatric patients aged 2 to less than 18 years of age, Treatment of moderate-to-severe plaque psoriasis in paediatric patients aged 6 to less than 18 years of age.

Day 30 discussion

Action: For discussion

Dermatology / Gastroenterology-Hepatology

3.1.25. inclisiran sodium - EMEA-002214-PIP01-17

Treatment of elevated cholesterol / indicated to lower LDL-C in adults and children aged 11 years old and older with heterozygous familial hypercholesterolemia in combination with other lipid lowering therapies., indicated to lower LDL-C in adults and children aged 11 years old and older with homozygous familial hypercholesterolemia in combination with other lipid lowering therapies.

Day 30 discussion

Action: For discussion

Endocrinology-Gynaecology-Fertility-Metabolism

3.1.26. [Anti-Mucosal Addressin Cell Adhesion Molecule Antibody - EMEA-002218-PIP01-17](#)

Treatment of Ulcerative Colitis, Treatment of Crohn's Disease / Treatment of moderate to severe active Crohn's Disease, Treatment of moderate to severe active Ulcerative Colitis

Day 30 discussion

Action: For discussion

Gastroenterology-Hepatology

3.1.27. [Ibrutinib - Orphan - EMEA-001397-PIP04-17](#)

Janssen-Cilag International N.V.; Treatment of cGVHD / indicated for the treatment of cGVHD in children 1 year of age and older.

Day 30 discussion

Action: For discussion

Immunology-Rheumatology-Transplantation

3.1.28. [Branaplam - EMEA-002204-PIP01-17](#)

Spinal Muscular Atrophy

Day 30 discussion

Action: For discussion

Neurology

3.1.29. [Opicinumab - EMEA-002194-PIP01-17](#)

Treatment of Multiple Sclerosis

Day 30 discussion

Action: For discussion

Neurology

3.1.30. [Glasdegib maleate - EMEA-002199-PIP01-17](#)

Treatment of acute myeloid leukaemia (AML) /as monotherapy for prevention of AML relapse in children aged 2 years up to <18 years with high risk for relapse post-alloSCT; • in combination with FLAG/DNX as reinduction treatment of R/R AML in children aged 2 years up to <18 years, followed by consolidation therapy with or without SCT, and finally single-agent glasdegib post-consolidation.

Day 30 discussion

Action: For discussion

Oncology

3.1.31. [isatuximab - Orphan - EMEA-002205-PIP01-17](#)

Sanofi-Aventis Recherche & Développement; Treatment of malignant neoplasms of the haematopoietic and lymphoid tissue / Treatment of relapsed, refractory acute lymphoblastic leukemia in combination with standard treatment in paediatric patients with no more than one prior salvage therapy, Treatment of relapsed, refractory acute myeloblastic leukemia in combination with standard treatment in paediatric patients with no more than one prior salvage therapy

Day 30 discussion

Action: For discussion

Oncology

3.1.32. [resminostat - Orphan - EMEA-002211-PIP01-17](#)

4SC AG; Cutaneous T-Cell Lymphoma

Day 30 discussion

Action: For discussion

Oncology

3.1.33. [EMEA-002197-PIP01-17](#)

Treatment of ocular melanoma

Day 30 discussion

Action: For discussion

Oncology

3.1.34. [EMEA-002217-PIP01-17](#)

Treatment of cartilage disorders

Day 30 discussion

Action: For discussion

Other

3.1.35. [Tolonium chloride - EMEA-002170-PIP01-17](#)

Dental and oral soft tissue infections

Day 30 discussion

Action: For discussion

Other

3.1.36. EMEA-001976-PIP01-16

Asthma / Once-daily maintenance treatment of asthma as prophylactic therapy in patients aged 12 years and older (and in age 5-11) where use of a maintenance anti-inflammatory medication is appropriate

Day 30 discussion

Action: For discussion

Pneumology - Allergology

3.1.37. Human Neutrophil Elastase Inhibitor - EMEA-002196-PIP01-17

Treatment of Non Cystic Fibrosis Bronchiectasis

Day 30 discussion

Action: For discussion

Pneumology - Allergology

3.1.38. ivacaftor / tezacaftor - EMEA-002191-PIP01-17

Treatment of Cystic Fibrosis

Day 30 discussion

Action: For discussion

Pneumology - Allergology

3.1.39. EMEA-002222-PIP01-17

Treatment of schizophrenia / Treatment of negative symptoms of schizophrenia

Day 30 discussion

Action: For discussion

Psychiatry

3.1.40. Plant-derived Quadrivalent VLP Influenza vaccine - EMEA-002220-PIP01-17

prophylaxis of seasonal influenza / For active immunization of persons six months of age and older for the prevention of disease caused by influenza A subtype viruses and type B viruses covered by the vaccine.

Day 30 discussion

Action: For discussion

Vaccines

3.1.41. EMEA-002215-PIP01-17

Disease caused by Streptococcus pneumoniae

Day 30 discussion

Action: For discussion

Vaccines

3.2. Discussions on Compliance Check

The following compliance checks have been put up for discussion and the members of the PDCO have been invited to comment on issues of possible non-compliance

3.2.1. rivaroxaban - EMEA-C4-000430-PIP01-08-M10

Bayer AG; Treatment of thromboembolic events

Day 30 discussion

Action: For discussion

Cardiovascular Diseases

3.2.2. rabeprazole (sodium) - EMEA-C-000055-PIP01-07-M05

Eisai Ltd.; Treatment of gastro-oesophageal reflux disease

Day 30 discussion

Action: For discussion

Gastroenterology-Hepatology

3.2.3. turoctocog alfa pegol - EMEA-C1-001174-PIP02-12-M02

Novo Nordisk A/S; Treatment of hereditary Factor VIII deficiency

Day 30 discussion

Action: For discussion

Haematology-Hemostaseology

3.2.4. Treosulfan - EMEA-C1-000883-PIP01-10-M04

medac Gesellschaft für klinische Spezialpräparate mbH; Conditioning treatment prior to

haematopoietic progenitor cell transplantation

Day 30 discussion

Action: For discussion

Immunology-Rheumatology-Transplantation / Oncology

3.2.5. [cannabidiol - EMEA-C1-001964-PIP01-16](#)

GW Research Ltd; Treatment of seizures associated with Infantile Spasms (IS)

Day 30 discussion

Action: For discussion

Neurology

3.2.6. [Autologous CD34+ enriched cell fraction that contains CD34+ cells transduced with lentiviral vector that encodes for the human ARSA cDNA sequence - EMEA-C1-001765-PIP02-15-M01](#)

Glaxo Smith Kline Trading Services Limited; Treatment of metachromatic leukodystrophy (MLD)

Day 30 discussion

Action: For discussion

Other

3.2.7. [A/\(H3N2\)-like virus antigen / B/Victoria-like virus antigen / B/Yamagata-like virus antigen / A/\(H1N1\)-like virus antigen - EMEA-C1-002068-PIP01-16](#)

Seqirus UK Limited; Prevention of Influenza

Day 30 discussion

Action: For discussion

Vaccines

3.3. **Discussions on Modification of an Agreed Paediatric Investigation Plan**

3.3.1. [Entolimod - EMEA-002020-PIP01-16-M01](#)

Cleveland BioLabs Inc; Treatment of acute Radiation Syndrome / Entolimod is indicated for reducing the risk of death following exposure to potentially lethal irradiation occurring as the results of a radiation disaster

Day 0 discussion

Action: For discussion

Other

3.3.2. Azilsartan medoxomil - EMEA-000237-PIP01-08-M07

Takeda Development Centre (Europe) Ltd.; Treatment of hypertension / Essential (primary) hypertension, Secondary hypertension

Day 30 discussion

Action: For discussion

Cardiovascular Diseases

3.3.3. Tolvaptan - EMEA-001231-PIP02-13-M05

Otsuka Pharmaceutical Europe Ltd.; Polycystic Kidney Disease (PKD), Dilutional hyponatraemia / Treatment of chronic (>48 hours) dilutional hyponatraemia resistant to fluid restriction (i.e., euvolemic and hypervolemic hyponatremia) associated with heart failure, cirrhosis or SIADH., Treatment of progression of ADPKD, Treatment of progression of ARPKD

Day 30 discussion

Action: For discussion

Endocrinology-Gynaecology-Fertility-Metabolism

3.3.4. Teduglutide ([gly2] recombinant human glucagon-like peptide) - Orphan - EMEA-000482-PIP01-08-M04

Shire Pharmaceuticals Ireland Limited; ICD-9-CM Diagnosis 579.3 - Other and unspecified post surgical non absorption - Syndrome Short Bowel / Treatment of Short Bowel Syndrome

Day 30 discussion

Action: For discussion

Gastroenterology-Hepatology

3.3.5. Fc- and CDR-modified humanised monoclonal antibody against C5 - Orphan - EMEA-002077-PIP01-16-M01

Alexion Europe SAS; Treatment of Paroxysmal Nocturnal Haemoglobinuria

Day 30 discussion

Action: For discussion

Haematology-Hemostaseology

3.3.6. sarilumab - EMEA-001045-PIP01-10-M01

sanofi-aventis recherche et développement; Treatment of chronic idiopathic arthritis (including rheumatoid arthritis, spondylarthritis, psoriatic arthritis and juvenile idiopathic

arthritis)

Day 30 discussion

Action: For discussion

Immunology-Rheumatology-Transplantation

3.3.7. Secukinumab - EMEA-000380-PIP01-08-M04

Novartis Europharm Ltd; Psoriasis / Moderate to severe plaque psoriasis in adults who are candidates for systemic therapy

Day 30 discussion

Action: For discussion

Immunology-Rheumatology-Transplantation

3.3.8. Upadacitinib - EMEA-001741-PIP01-14-M01

AbbVie Ltd; Treatment of Chronic Idiopathic Arthritis / Treatment of Juvenile Idiopathic Arthritis

Day 30 discussion

Action: For discussion

Immunology-Rheumatology-Transplantation

3.3.9. Adalimumab - EMEA-000366-PIP02-09-M05

AbbVie Limited; Ulcerative Colitis / Treatment of Moderate to severe ulcerative colitis

Day 30 discussion

Action: For discussion

Immunology-Rheumatology-Transplantation / Ophthalmology / Dermatology / Gastroenterology-Hepatology

3.3.10. 4-amino-1-[5-chloro-2,5-dideoxy-2-fluoro-3-O-(2-methylpropanoyl)-4-[[2-methylpropanoyl]oxy]methyl]- α -L-lyxofuranosyl]-2(1H)-pyrimidinone - EMEA-001758-PIP01-15-M02

Janssen-Cilag International NV; Treatment of lower respiratory tract disease caused by human respiratory syncytial virus

Day 30 discussion

Action: For discussion

Infectious Diseases

3.3.11. Telavancin hydrochloride - EMEA-000239-PIP01-08-M03

Theravance Biopharma Ireland Ltd.; Nosocomial Pneumonia (NP)

Day 30 discussion

Action: For discussion

Infectious Diseases

3.3.12. Thrombomodulin alfa - EMEA-001363-PIP01-12-M01

Asahi Kasei Pharma America Corporation; Treatment of sepsis / Treatment of patients with severe sepsis (respiratory failure and/or septic shock) with coagulopathy

Day 30 discussion

Action: For discussion

Infectious Diseases

3.3.13. Bumetanide - EMEA-001303-PIP01-12-M02

Les Laboratoires Servier; Treatment of Autism Spectrum Disorder

Day 30 discussion

Action: For discussion

Neurology

3.3.14. eculizumab - Orphan - EMEA-000876-PIP03-14-M01

Alexion Europe SAS; Neuromyelitis optica spectrum disorders / Treatment of Relapsing Neuromyelitis Optica Spectrum Disorders in the paediatric population

Day 30 discussion

Action: For discussion

Neurology

3.3.15. lacosamide - EMEA-000402-PIP03-17-M01

UCB Pharma S.A.; Treatment of Generalized Epilepsy and Epilepsy Syndromes: Epilepsy - generalized idiopathic epilepsy and epilepsy syndromes [G40.3] Epilepsy - Other generalized epilepsy and epileptic syndromes [G40.4] / Adjunctive therapy in the treatment of primary generalized tonic-clonic seizures (PGTCS) in pediatric patients with idiopathic generalized epilepsy (IGE)(4 years to <18 years), Adjunctive therapy in the treatment of epileptic syndromes associated with generalized seizures in pediatric patients with epilepsy birth to <18 years (specific epileptic syndrome(s) to be based on future clinical development further to exploratory study results)

Day 30 discussion

Action: For discussion

Neurology

3.3.16. ozanimod - EMEA-001710-PIP02-14-M02

Celgene Europe Limited; Treatment of multiple sclerosis / Treatment of relapsing remitting forms of multiple sclerosis

Day 30 discussion

Action: For discussion

Neurology

3.3.17. daunorubicin (liposomal combination) / cytarabine (liposomal combination) - Orphan - EMEA-001858-PIP02-16-M01

JAZZ PHARMACEUTICALS IRELAND LIMITED; Acute Myeloid Leukemia

Day 30 discussion

Action: For discussion

Oncology

3.3.18. avelumab (recombinant human monoclonal IgG1 antibody directed against Programmed Death Ligand-1 (anti-PD-L1); - Orphan - EMEA-001849-PIP02-15-M01

Merck KGaA; Treatment of all conditions included in the category of solid malignant neoplasms (including central nervous system tumours and lymphoma)

Day 30 discussion

Action: For discussion

Oncology

3.3.19. Naltrexone HCl / Bupropion HCl - EMEA-001373-PIP01-12-M03

Orexigen Therapeutics Ireland Limited; Treatment of obesity

Day 30 discussion

Action: For discussion

Other

3.3.20. Palovarotene - Orphan - EMEA-001662-PIP01-14-M01

Clementia Pharmaceuticals Inc.; Treatment of Fibrodysplasia Ossificans Progressiva (FOP)

Day 30 discussion

Action: For discussion

Other

3.3.21. Tapentadol - EMEA-000018-PIP01-07-M14

Grünenthal GmbH; Treatment of acute pain

Day 30 discussion

Action: For discussion

Pain

3.3.22. mirabegron - EMEA-000597-PIP02-10-M06

Astellas Pharma Europe B.V.; Treatment of idiopathic overactive bladder / Symptomatic treatment of urge incontinence and/or increased urinary frequency and urgency as may occur in patients with overactive bladder syndrome

Day 30 discussion

Action: For discussion

Uro-nephrology

4. Nominations

Disclosure of information related to this section cannot be released at the present time as it is deemed to contain commercially confidential information.

4.1. List of letters of intent received for submission of applications with start of procedure 28 November 2017 for Nomination of Rapporteur and Peer reviewer

Action: For adoption

4.2. Nomination of Rapporteur for requests of confirmation on the applicability of the EMA decision on class waiver.

Action: For adoption

4.3. Nominations for other activities

Action: For adoption

5. Scientific Advice Working Party (SAWP) and Paediatric Committee (PDCO) Interaction

Disclosure of information related to this section cannot be released at the present time as it is deemed to contain commercially confidential information.

6. Discussion on the applicability of class waivers

Disclosure of some information related to this section cannot be released at the present time as it is deemed to contain commercially confidential information.

6.1. Discussions on the applicability of class waiver for products

None

7. Discussion on the inclusion of an indication within a condition in an agreed PIP/waiver

7.1. Discussion on the possibility to include an indication within a condition in an agreed PIP/waiver

7.1.1. Fully human anti-human PCSK9 monoclonal antibody - EMEA-001169-PIP01-11

sanofi-aventis R&D; Treatment of elevated cholesterol/Reduction in the frequency of apheresis in HeFH patients

Action: For adoption

8. Annual reports on deferrals

Note: The annual reports on deferrals to be noted by the members of the PDCO are flagged in the Annex B.

9. Organisational, regulatory and methodological matters

9.1. Mandate and organisation of the PDCO

None

9.2. Coordination with EMA Scientific Committees or CMDh-v

9.2.1. Committee for Medicinal Products for Human Use (CHMP)

Action: For information

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

9.3.1. Non-clinical Working Group: D30 Products identified

PDCO member: Karen van Malderen

Action: For information

9.3.2. Formulation Working Group

PDCO member: Brian Aylward

Action: For information

9.3.3. CMDh letter to PDCO after Art. 29 referral – Ozanex (ozenoxacin)

PDCO member: Marianne Orholm, Maria Fernandez Cortizo

Action: For adoption

9.3.4. Guideline on clinical investigation of medicinal products in the treatment of epileptic disorders

PDCO member: Sylvie Benchetrit

Action: For discussion

9.3.5. Draft agenda PCWP/HCPWP joint meeting to be held at EMA on 20 September 2017

Action: For information

9.3.6. Draft Agenda - Session on antimicrobial resistance EMA Working Parties with Patients' and PCWP and HCPWP joint meeting to be held at EMA on 19 September 2017

Action: For information

9.3.7. Reflection paper on the use of extrapolation in paediatric medicines development

PDCO member: Dirk Mentzer

Action: For comments (adoption planned for October)

9.3.8. Call for membership of the Patients and Consumers Working Party and Healthcare Professional's Working Party

PDCO member: Dirk Mentzer

Action: For adoption

9.3.9. Paediatric Addendum to the guideline on the evaluation of medicinal products indicated for treatment of bacterial infections

PDCO members: Maria Fernandez Cortizo, Irja Lutsar, Marianne Orholm

Action: For adoption

9.4. **Cooperation within the EU regulatory network**

None

9.5. **Cooperation with International Regulators**

None

9.6. **Contacts of the PDCO with external parties and interaction with the Interested Parties to the Committee**

None

9.7. **PDCO work plan**

None

9.8. **Planning and reporting**

9.8.1. Presentation regarding T cells expressing Chimeric Antigen Receptors (CAR-Ts)

Action: For discussion

9.8.2. Business Pipeline Report - Forecast for 2017 - Update Q3/2017

Action: Tabled for information

10. Any other business

10.1. Training for PDCO members and alternates

11. Breakout sessions

11.1.1. Paediatric oncology

Action: For discussion on Thursday, 14:00 - 15:00, room 3H

11.1.2. Neonatology

Action: For discussion on Thursday, 14:00 - 15:00, room 3H

11.1.3. Inventory

Action: For discussion on Thursday, 14:00 - 15:00, delegates' lounge

12. Explanatory notes

The Notes give a brief explanation of relevant agenda items and should be read in conjunction with the agenda.

Paediatric investigation plan (PIP) (section 2.1 Opinion on PIPs and section 3.1 Discussions on PIPs)

A paediatric investigation plan (PIP) is a development plan aimed at ensuring that the necessary data are obtained through studies in children, when it is safe to do so, to support the authorisation of a medicine for children. Pharmaceutical companies submit proposals for PIPs to the European Medicines Agency's Paediatric Committee (PDCO). This Committee is responsible for agreeing or refusing the plan.

Compliance checks (section 2.2 Opinions on Compliance check, section 3.2 Discussions on Compliance check)

A compliance check may be necessary before any application for marketing authorisation (even for an adult indication) can be considered valid, if there was no deferral for at least one of the studies agreed in the PIP, or after the due date of initiation or completion of a study/measure. The same applies to some regulatory applications for authorised products, as described above.

Modification of an Agreed Paediatric Investigation Plan (section 2.3 Opinions on Modification of an agreed PIP, section 3.3 Discussions on Modification of an agreed PIP)

The development plan for a medicine can be modified at a later stage as knowledge increases. Modifications can also be made if the applicant encounters such difficulties with the implementation of a PIP, which render it unworkable or no longer appropriate.

In some cases, studies can be deferred until after the studies in adults have been conducted. This ensures that research in children is done only when it is safe and ethical to do so. Even when studies are deferred, the PIP will include details of the paediatric studies and their timelines.

Class waiver (section 6 Discussion on the applicability of class waiver)

As some diseases do not affect children (for example Parkinson's disease), the development of medicines for these diseases should not be performed in children. In these cases, a PIP is not required and it will be waived. For more information on the classes of diseases subject to waivers, see [class waivers](#).

Annual reports on deferrals (section 8)

If the medicinal product is approved in the EU, annual reports on the deferred measures in the PIP must be submitted to the Agency.

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