19 June 2017
EMA/331620/2017
Inspections, Human Medicines Pharmacovigilance and Committees Division

Paediatric Committee (PDCO)

Draft agenda for the meeting on 20-23 June 2017

Chair: Dirk Mentzer – Vice-Chair: Koenraad Norga

20 June 2017, 14:00 - 19:00, room 3A

21 June 2017, 08:30 - 19:00, room 3A

22 June 2017, 08:30 - 19:00, room 3A

23 June 2017, 08:30 - 13:00, room 3A

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 20-23 June 2017. See June 2017 PDCO minutes (to be published post July 2017 PDCO meeting).

1.2. Adoption of agenda

PDCO agenda for 20-23 June 2017.

1.3. Adoption of the minutes

PDCO minutes for 16-19 May 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. Methacholine Chloride - EMEA-002120-PIP01-17

Diagnosis of asthma

Day 60 opinion

Action: For adoption

Diagnostic

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

Alexion Europe SAS; Treatment of Paroxysmal Nocturnal Haemoglobinuria

Day 120 opinion

Action: For adoption

Haematology-Hemostaseology

2.1.3. tocilizumab - EMEA-000309-PIP04-17

Treatment of SSc (ICD 10-M34)/scleroderma and associated disorders (MedDRA)/ Treatment of juvenile Systemic Sclerosis (jSSc) in children 5 years of age and older

Day 60 opinion

Action: For adoption

Immunology-Rheumatology-Transplantation

2.1.4. Daunorubicin (liposomal combination) / Cytarabine (liposomal combination) - Orphan - EMEA-001858-PIP02-16

Jazz Pharmaceuticals Ireland Limited; Acute myeloid leukemia

Day 120 opinion

Action: For adoption

Oncology

2.1.5. Venetoclax - Orphan - EMEA-002018-PIP02-16

AbbVie Ltd; Treatment of haematopoietic and lymphoid malignant neoplasms, Treatment of solid tumour malignant neoplasms / As monotherapy, or in combination with chemotherapy, for the treatment of relapsed or refractory NHL patients < 18 years of age, who have progressed following autologous stem cell transplantation or who are ineligible for transplantation, As monotherapy or in combination for the treatment of patients with relapsed or refractory neuroblastoma < 18 years of age, As monotherapy, or in combination with chemotherapy, for the treatment of relapsed or refractory ALL in the third line setting in patients < 18 years of age, As monotherapy, or in combination with chemotherapy, for the treatment of relapsed or refractory AML in patients < 18 years of age

Day 120 opinion

Action: For adoption

Oncology / Haematology-Hemostaseology

2.1.6. Influenza virus surface antigens (haemagglutinin and neuraminidase) of strain B (Victoria lineage) / Influenza virus surface antigens (haemagglutinin and neuraminidase) of strain B (Yamagata lineage) / Influenza virus surface antigens (haemagglutinin and neuraminidase) of strain A (H3N2) / Influenza virus surface antigens (haemagglutinin and neuraminidase) of strain A (H1N1) - EMEA-002068-PIP01-16

Prevention of influenza

Day 120 opinion

Action: For adoption

Vaccines

2.1.7. EMEA-002148-PIP01-17

Treatment of venous and mixed (venous/arterial) leg ulcers

Day 60 opinion

Action: For adoption

Cardiovascular Diseases

2.1.8. macitentan - Orphan - EMEA-001032-PIP02-17

Actelion Registration Ltd.; Chronic Thromboembolic Pulmonary Hypertension (CTEPH)

Day 60 opinion

Action: For adoption

Cardiovascular Diseases

2.1.9. Empagliflozin - EMEA-000828-PIP05-17

Prevention of cardiovascular events in patients with chronic heart failure

Day 60 opinion

Action: For adoption

Endocrinology-Gynaecology-Fertility-Metabolism / Cardiovascular Diseases

2.1.10. H-Tyr-Gly-Arg-Lys-Lys-Arg-Arg-Gln-Arg-Arg-Arg-Lys-Leu-Ser-Ser-Ile-Glu-Ser-Asp-Val-OH (YGRKKRRQRRRKLSSIESDV) - EMEA-002108-PIP01-16

Acute Ischemic Stroke (AIS) in adult subjects with a large intracranial arterial occlusion, a small ischemic core, and good collaterals

Day 60 opinion

Action: For adoption

Neurology

2.1.11. Recombinant humanized anti-alpha-synuclein IgG1 monoclonal antibody - EMEA-002137-PIP01-17

treatment of Parkinson's disease (in adults)

Day 60 opinion

Action: For adoption

Neurology

2.1.12. OSIMERTINIB MESYLATE - EMEA-002125-PIP01-17

Treatment of lung carcinoma (small cell and non-small cell carcinoma)

Day 60 opinion

Action: For adoption

Oncology

2.1.13. Pexastimogene devacirepvec - Orphan - EMEA-002124-PIP01-17

Transgene S.A.; Treatment of hepatocellular carcinoma (MedDra PT: 10073071)

Day 60 opinion

Action: For adoption

Oncology

2.1.14. daxibotulinumtoxinA - EMEA-002149-PIP01-17

Treatment for temporary improvement in the appearance of moderate to severe glabellar lines with corrugator and/or procerus muscle activity in adults

Day 60 opinion

Action: For adoption

Other

2.1.15. Diclofenac sodium - EMEA-002132-PIP01-17

Symptomatic relief of pain associated with osteoarthritis, Symptomatic relief of mild to moderate pain and inflammation / Indicated for the symptomatic relief of pain associated with osteoarthritis in superficial joints, including the knee., For the local symptomatic relief of mild to moderate pain and inflammation following acute blunt trauma of small and medium-sized joints and periarticular structures, such as trauma of the tendons, ligaments, muscles and joints e.g. due to sprains and strains.

Day 60 opinion

Action: For adoption

Pain

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. Raltegravir - EMEA-C-000279-PIP01-08-M05

Merck Sharp & Dohme (Europe) Inc., Treatment of Human Immunodeficieny Virus (HIV-1) infection

Opinion adopted via written procedure on 19 June 2017

Action: For information

Infectious Diseases

2.2.2. Meropenem trihydrate / Vaborbactam - EMEA-C1-001731-PIP01-14

Rempex Pharmaceuticals, Treatment of Gram-negative bacterial infections

Day 30 letter

Action: For adoption

Infectious Diseases

2.2.3. Meropenem trihydrate / Vaborbactam - EMEA-C1-001740-PIP01-14

Rempex Pharmaceuticals, Treatment of Gram-negative bacterial infections

Day 30 letter

Action: For adoption

Infectious Diseases

2.2.4. Tofacitinib - EMEA-C1-000576-PIP03-12

Pfizer Limited; Treatment of Ulcerative Colitis

Day 60 letter

Action: For adoption

Immunology-Rheumatology-Transplantation

2.2.5. Human normal immunoglobulin - EMEA-C1-001797-PIP01-15

Octapharma Pharamzeutika Produktionsges.m.b.H; Treatment of primary immunodeficiency

Day 60 letter

Action: For adoption

Immunology-Rheumatology-Transplantation / Haematology-Hemostaseology

2.2.6. Ibrutinib - EMEA-C1-001397-PIP03-14-M02

Janssen-Cilag International N.V.; Treatment of mature B-cell neoplasm

Day 60 letter

Action: For adoption

Oncology

2.3. Opinions on Modification of an Agreed Paediatric Investigation Plan

2.3.1. Apremilast - Orphan - EMEA-000715-PIP05-13-M01

Celgene Europe Limited; Treatment of Behcets Disease / Treatment of patients with active oral ulcers (with or without genital ulcers) associated with Behcets Disease, who are candidates for systemic therapy

Revised opinion adopted via written procedure on 14 June 2017

Action: For information

Immunology-Rheumatology-Transplantation

2.3.2. apixaban - EMEA-000183-PIP01-08-M05

Bristol-Myers Squibb / Pfizer EEIG; Prevention of arterial thromboembolism, Prevention of venous thromboembolism / Prevention of venous thromboembolism (VTE) in paediatric subjects (1 to <18 years old) with a newly diagnosed acute lymphoblastic leukemia (ALL) or lymphoma (T or B cell), a functioning central venous access device (CVAD) and receiving PEG L-asparaginase during chemotherapy induction. Prevention of TE in paediatric patients (birth to below 18 years old) with cardiac disease.

Day 60 opinion

Action: For adoption

Cardiovascular Diseases

2.3.3. Allantoin - Orphan - EMEA-001590-PIP01-13-M04

Scioderm, Inc.; Treatment of epidermolysis bullosa

Day 60 opinion

Action: For adoption

Dermatology

2.3.4. Asfotase alfa - Orphan - EMEA-000987-PIP01-10-M03

Alexion Europe SAS; Treatment of hypophosphatasia

Day 60 opinion

Action: For adoption

Endocrinology-Gynaecology-Fertility-Metabolism

2.3.5. Canagliflozin - EMEA-001030-PIP01-10-M07

Janssen-Cilag International NV; Treatment of Type 2 Diabetes Mellitus

Day 60 opinion

Action: For adoption

Endocrinology-Gynaecology-Fertility-Metabolism

2.3.6. Liraglutide - EMEA-000128-PIP01-07-M08

Novo Nordisk A/S; Treatment of type 2 Diabetes Mellitus

Day 60 opinion

Action: For adoption

Endocrinology-Gynaecology-Fertility-Metabolism

2.3.7. semaglutide - EMEA-001441-PIP02-15-M01

Novo Nordisk; Type 2 Diabetes Mellitus

Day 60 opinion

Action: For adoption

Endocrinology-Gynaecology-Fertility-Metabolism

2.3.8. Sodium zirconium cyclosilicate - EMEA-001539-PIP01-13-M02

AstraZeneca AB; Treatment of Hyperkalaemia

Day 60 opinion

Action: For adoption

Endocrinology-Gynaecology-Fertility-Metabolism

2.3.9. sotagliflozin - EMEA-001517-PIP01-13-M01

sanofi-aventis R&D; Treatment of type 2 diabetes mellitus

Day 60 opinion

Action: For adoption

Endocrinology-Gynaecology-Fertility-Metabolism

2.3.10. sotagliflozin - EMEA-001517-PIP02-14-M01

sanofi-aventis R&D; Treatment of type 1 diabetes mellitus

Day 60 opinion

Action: For adoption

Endocrinology-Gynaecology-Fertility-Metabolism

2.3.11. Obeticholic Acid (6 alpha-ethylchenodeoxycholic acid) - Orphan - EMEA-001304-PIP02-13-M03

Intercept Pharma Ltd.; Primary Biliary Cirrhosis (PBC) / Biliary Atresia

Day 60 opinion

Action: For adoption

Gastroenterology-Hepatology

2.3.12. Coagulation Factor VIIa (Recombinant) - EMEA-001203-PIP02-14-M02

LFB SA; Treatment of congenital coagulation disorders, Treatment of acquired haemophilia / Treatment of bleeding and prevention of bleeding in those undergoing surgery or invasive procedures in patients with haemophilia A or B with inhibitors to Factors VIII or IX, Treatment of bleeding and prevention of bleeding in those undergoing surgery or invasive procedures in patients with acquired haemophilia

Day 60 opinion

Action: For adoption

Haematology-Hemostaseology

2.3.13. Damoctocog alfa pegol - Orphan - EMEA-001229-PIP01-11-M03

Bayer AG; Treatment of hereditary factor VIII deficiency / Treatment and prophylaxis of bleeding in patients with haemophilia A (hereditary factor VIII deficiency).

Day 60 opinion

Action: For adoption

Haematology-Hemostaseology

2.3.14. Luspatercept - Orphan - EMEA-001521-PIP01-13-M01

Celgene Europe Ltd; Treatment of myelodysplastic syndromes, Treatment of beta-thalassaemia, Treatment of anaemia in patients with b-thalassaemia intermedia and major

Day 60 opinion

Action: For adoption

Haematology-Hemostaseology

2.3.15. Recombinant fusion protein linking coagulation factor IX with albumin - Orphan - EMEA-001107-PIP01-10-M03

CSL Behring GmbH; Treatment of hereditary factor IX deficiency

Day 60 opinion

Action: For adoption

Haematology-Hemostaseology

2.3.16. Recombinant Fusion Protein Linking Coagulation Factor VIIa with Albumin (rVIIa-FP) - Orphan - EMEA-001886-PIP01-15-M01

CSL Behring GmbH; Treatment of Haemophilia B, Treatment of Haemophilia A / Treatment of Haemophilia B with Inhibitors, Treatment of Haemophilia A with Inhibitors

Day 60 opinion

Action: For adoption

Haematology-Hemostaseology

2.3.17. Recombinant Fusion Protein Linking Coagulation Factor VIIa with Albumin (rVIIa-FP) - Orphan - EMEA-001886-PIP02-15-M01

CSL Behring GmbH; Treatment of congenital Factor VII Deficiency / Treatment of congenital Factor VII Deficiency

Day 60 opinion

Action: For adoption

Haematology-Hemostaseology

2.3.18. Romiplostim - Orphan - EMEA-000653-PIP01-09-M05

Amgen Europe B.V.; Treatment of disease-related thrombocytopenia in myelodysplastic syndrome, Treatment of immune thrombocytopenia (idiopathic thrombocytopenic purpura) / Treatment of chronic immune thrombocytopenia (idiopathic thrombocytopenic purpura; ITP) in paediatric patients who are refractory or intolerant to other treatments (e.g., glucocorticosteroids, immunoglobulins, splenectomy)

Day 60 opinion

Action: For adoption

Haematology-Hemostaseology

2.3.19. Treosulfan - Orphan - EMEA-000883-PIP01-10-M04

medac Gesellschaft für klinische Spezialpräparate mbH; Conditioning treatment prior to haematopoietic progenitor cell transplantation

Day 60 opinion

Action: For adoption

Immunology-Rheumatology-Transplantation / Oncology

2.3.20. Lamivudine (3TC) / Abacavir (ABC) / Dolutegravir (DTG) - EMEA-001219-PIP01-11-M03

ViiV Healthcare UK Limited; Treatment of Human Immunodeficiency Virus (HIV-1) infection / Treatment Human Immunodeficiency Virus (HIV-1) infection in paediatric population

Day 60 opinion

Action: For adoption

Infectious Diseases

2.3.21. Tenofovir Alafenamide / Emtricitabine / Cobicistat / Elvitegravir - EMEA-001460-PIP01-13-M02

Gilead Sciences International Ltd.; Treatment of human immunodeficiency virus (HIV-1) infection / indicated for the treatment of HIV-1 infection in paediatric patients from 6 years to less than 18 years.

Day 60 opinion

Action: For adoption

Infectious Diseases

2.3.22. zanamivir - EMEA-001318-PIP01-12-M02

GlaxoSmithKline Trading Services Limited; Treatment of influenza, Prevention of influenza / Treatment of influenza A and B virus infection, Prevention of influenza A and B virus infection

Day 60 opinion

Action: For adoption: Oral Explanation Meeting to be held on 21 Wednesday 2017, 16:00-17:00

Infectious Diseases

2.3.23. Bivalent anti-human myostatin adnectin recombinant human IgG1-Fc fusion protein - EMEA-001793-PIP01-15-M02

Bristol-Myers Squibb International Corporation; Treatment of Duchenne Muscular Dystrophy / Treatment of Duchenne Muscular Dystrophy in patients from 2 to less than 18 years of age

Day 60 opinion

Action: For adoption

Neurology

2.3.24. Brentuximab vedotin - Orphan - EMEA-000980-PIP01-10-M05

Takeda Pharma A/S; Treatment of Hodgkin lymphoma / Treatment of paediatric patients with newly diagnosed, relapsed or refractory Hodgkin lymphoma (from 5 years of age)

Day 60 opinion

Action: For adoption

Oncology

2.3.25. decitabine - Orphan - EMEA-000555-PIP01-09-M06

Janssen-Cilag International NV; Treatment of acute myeloid leukemia / Treatment of paediatric patients with acute myeloid leukaemia who have high-risk cytogenetics, or are refractory to, or have a relapse after first-line treatment

Day 60 opinion

Action: For adoption

Oncology

2.3.26. Isopropyl Alcohol / Chlorhexidine Gluconate - EMEA-000989-PIP01-10-M02

3M Health Care Limited; Prevention of infection

Day 60 opinion

Action: For adoption

Other

2.3.27. ivacaftor / lumacaftor - EMEA-001582-PIP01-13-M06

Vertex Pharmaceuticals (Europe) Limited; Treatment of cystic fibrosis

Day 60 opinion

Action: For adoption

Other

2.3.28. Recombinant Varicella Zoster Virus (VZV) glycoprotein E - EMEA-001426-PIP01-13-M01

GlaxoSmithKline Biologicals SA; Prevention of Varicella Zoster Virus reactivation / Prevention of herpes zoster in immunocompromised subjects aged 1 to 17 years

Day 60 opinion

Action: For adoption

Vaccines

2.4. Opinions on Re-examinations

2.4.1. rivaroxaban - EMEA-000430-PIP01-08-M10

Bayer Pharma AG; Treatment of thromboembolic events, Prevention of thromboembolic events, Treatment (secondary prevention) of venous thromboembolism

Day 30 opinion

Action: For adoption; Oral Explanation Meeting to be held on Wednesday 21 June 2017, 14:00-15:00 UK time

Cardiovascular Diseases

2.4.2. Ivacaftor - EMEA-001640-PIP01-14-M02

Vertex Pharamceuticals (Europe) lTd; Treatment of Cystic Fibrosis

Day 30 opinion

Action: For adoption

Pneumology - Allergology

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. Birch bark extract - Orphan - EMEA-001299-PIP02-16

Birken AG; Treatment of epidermolysis bullosa

Day 90 discussion

Action: For discussion

Dermatology

3.1.2. Selonsertib - EMEA-001868-PIP03-16

K75.8 Other specified inflammatory liver diseases (non-alcoholic steatohepatitis [NASH]) / Treatment of Non-Alcoholic Steatohepatitis (NASH) with moderate to severe fibrosis (F2-F4)

in paediatric subjects, 8 to < 18 years of age

Day 90 discussion

Action: For discussion

Gastroenterology-Hepatology

3.1.3. Atacicept - EMEA-002004-PIP01-16

Treatment of systemic lupus erythematosus

Day 90 discussion

Action: For discussion

Immunology-Rheumatology-Transplantation

3.1.4. Lefamulin - EMEA-002075-PIP01-16

Treatment of community-acquired pneumonia

Day 90 discussion

Action: For discussion

Infectious Diseases

3.1.5. fenfluramine hydrochloride - Orphan - EMEA-001990-PIP01-16

Zogenix International Ltd; Dravet syndrome / The adjunctive treatment of seizures in paediatric patients at least 1 year of age with Dravet syndrome

Day 90 discussion

Action: For discussion

Neurology

3.1.6. Sarizotan hydrochloride - Orphan - EMEA-001808-PIP02-16

Newron Pharmaceuticals SpA; Treatment of Rett syndrome

Day 90 discussion

Action: For discussion

Neurology

3.1.7. Autologous T cells transduced with retroviral vector encoding an anti-CD19 CD28/CD3-zeta chimeric antigen receptor - Orphan - EMEA-001862-PIP01-15

Kite Pharma EU B.V.; Treatment of B lymphoblastic leukaemia/lymphoma

Day 90 discussion

Action: For discussion

Oncology

3.1.8. Autologous T cells transduced with retroviral vector encoding an anti-CD19 CD28/CD3-zeta chimeric antigen receptor - Orphan - EMEA-002010-PIP01-16

Kite Pharma EU B.V.; Treatment of B-cell neoplasm

Day 90 discussion

Action: For discussion

Oncology

3.1.9. Recombinant protein derived from the saliva of Ornithodoros moubata tick - EMEA-002100-PIP01-16

Atypical haemolytic uraemic syndrome

Day 60 discussion

Action: For discussion

Haematology-Hemostaseology

3.1.10. Recombinant protein derived from the saliva of Ornithodoros moubata tick - Orphan - EMEA-002100-PIP02-16

Akari Therapeutics plc; Paroxysmal nocturnal haemoglobinuria

Day 60 discussion

Action: For discussion

Haematology-Hemostaseology

3.1.11. Cefiderocol - EMEA-002133-PIP01-17

Treatment of Gram-negative bacterial infections

Day 60 discussion

Action: For discussion

Infectious Diseases

3.1.12. 5-[4-[2-(5-(1-hydroxyethyl)-2-pyridinyl)ethoxy]benzyl]-2,4-thiazolidinedione hydrochloride - Orphan - EMEA-002106-PIP01-16

Minoryx Therapeutics SL; Treatment of adrenoleukodystrophy / Treatment of X-linked adrenoleukodystrophy

Day 60 discussion

Action: For discussion

Neurology

3.1.13. Adeno-associated viral vector serotype rh.10 carrying the human N-sulfoglucosamine sulfohydrolase cDNA - Orphan - EMEA-002122-PIP02-17

LYSOGENE; Mucopolysaccharidosis type IIIA

Day 60 discussion

Action: For discussion

Neurology

3.1.14. Entrectinib - Orphan - EMEA-002096-PIP01-16

Ignyta, Inc.; Treatment of all conditions included in the category of malignant neoplasms (except central nervous system tumours, haematopoietic and lymphoid tissue neoplasms), Treatment of malignant neoplasms of the central nervous system / Treatment of primary brain tumours with NTRK1/2/3, ROS1 or ALK gene fusions, Treatment of extracranial solid tumours with NTRK1/2/3, ROS1 or ALK gene fusions

Day 60 discussion

Action: For discussion

Oncology

3.1.15. Pevonedistat - EMEA-002117-PIP01-17

Acute Myeloid Leukemia (AML), Myelodysplastic Syndromes (MDS) / The treatment of paediatric patients with relapsed or refractory (R/R) MDS (including juvenile myelomonocytic leukemia), The treatment of paediatric patients with relapsed or refractory (R/R) AML

Day 60 discussion

Action: For discussion

Oncology

3.1.16. EMEA-002121-PIP01-17

Treatment of insomnia / Treatment of attention deficit hyperactivity disorder (ADHD)-related insomnia

Day 60 discussion

Action: For discussion

Psychiatry

3.1.17. Recombinant Clostridium difficile Toxoid B / Recombinant Clostridium difficile Toxoid A - EMEA-002112-PIP01-16

Prevention of Clostridium difficile infection (CDI) / Active immunization for the prevention of primary Clostridium difficile infection in children and adolescents 2 to 18 years of age

Day 60 discussion

Action: For discussion

Vaccines

3.1.18. tralokinumab - EMEA-001900-PIP02-17

Treatment of Atopic Dermatitis

Day 30 discussion

Action: For discussion

Dermatology

3.1.19. EMEA-002162-PIP01-17

type 2 diabetes mellitus

Day 30 discussion

Action: For discussion

Endocrinology-Gynaecology-Fertility-Metabolism

3.1.20. Maralixibat Chloride - Orphan - EMEA-001475-PIP03-17

Shire Pharmaceuticals Ireland Limited; Treatment of Progressive Familial Intrahepatic Cholestasis

Day 30 discussion

Action: For discussion

Gastroenterology-Hepatology

3.1.21. Non-Pathogenic Bacterial Lysate of Escherichia coli (DSM 17252) and Enterococcus faecalis (DSM 16440) - EMEA-002155-PIP01-17

Irritable bowel syndrome (IBS)

Day 30 discussion

Action: For discussion

Gastroenterology-Hepatology

3.1.22. Hydroxycarbamide - EMEA-002156-PIP01-17

Sickle Cell Syndrome

Day 30 discussion

Action: For discussion

Haematology-Hemostaseology

3.1.23. Risankizumab - EMEA-001776-PIP02-17

Chronic Idiopathic Arthritis

Day 30 discussion

Action: For discussion

Immunology-Rheumatology-Transplantation

3.1.24. Human normal immunoglobulin for intravenous use - EMEA-002163-PIP01-17

Replacement therapy: D80-D84 Primary Immunodeficiency Syndromes with failure of antibody production. Hypogammaglobulinaemia and recurrent bacterial infections in patients with chronic lymphocytic leukaemia, in whom prophylactic antibiotics have failed. Hypogammaglobulinaemia and recurrent bacterial infections in plateau phase multiple myeloma patients who have failed to respond to pneumococcal immunisation. Hypogammaglobulinaemia in patients after allogeneic haematopoietic stem cell transplantation (HSCT)

Day 30 discussion

Action: For discussion

Immunology-Rheumatology-Transplantation / Haematology-Hemostaseology

3.1.25. EMEA-002080-PIP01-16

Treatment of influenza

Day 30 discussion

Action: For discussion

Infectious Diseases

3.1.26. Obiltoxaximab - EMEA-002144-PIP01-17

Treatment of bacillary infection, Prevention of bacillary infection / Treatment of inhalation anthrax following exposure to Bacillus anthracis in combination with appropriate antibacterial drugs, Post-exposure prophylaxis of inhalation anthrax when alternative therapies are not available or are not appropriate, Prophylaxis of inhalation anthrax when alternative therapies are not available or are not appropriate

Day 30 discussion

Action: For discussion

Infectious Diseases

3.1.27. Insulin human - EMEA-002116-PIP01-17

Treatment of intestinal malabsorption in preterm infants

Day 30 discussion

Action: For discussion

Neonatology - Paediatric Intensive Care / Gastroenterology-Hepatology

3.1.28. Insulin human - Orphan - EMEA-002116-PIP02-17

Nutrinia, Ltd.; Short bowel syndrome / Treatment of infants with Short Bowel Syndrome following surgical resection to improve intestinal absorption of nutrients and fluids

Day 30 discussion

Action: For discussion

Neonatology - Paediatric Intensive Care / Gastroenterology-Hepatology

3.1.29. anetumab ravtansine - Orphan - EMEA-002123-PIP01-17

Bayer AG; Treatment of acute myeloid leukaemia, Treatment of mesothelioma, Treatment of patients from 2 to less than 18 years of age with relapsed and/or refractory mesothelin-positive acute myeloid leukaemia

Day 30 discussion

Action: For discussion

Oncology

3.1.30. Autologous T cells transduced with lentiviral vector containing a chimeric antigen receptor directed against CD19 - Orphan - EMEA-001654-PIP02-17

Novartis Europharm Limited; Mature B-cell neoplasm / Treatment of paediatric patients with relapsed or refractory mature B-cell non-Hodgkin's lymphoma

Day 30 discussion

Action: For discussion

Oncology

3.1.31. carotuximab - Orphan - EMEA-002138-PIP01-17

TRACON Pharma Limited; Treatment of angiosarcoma

Day 30 discussion

Action: For discussion

Oncology

3.1.32. daratumumab - Orphan - EMEA-002152-PIP01-17

Janssen-Cilag International N.V.; Acute Lymphoblastic Leukemia / Daratumumab in combination with standard chemotherapy is indicated for the treatment of pediatric patients aged 1 month to 18 years with acute lymphoblastic leukemia.

Day 30 discussion

Action: For discussion

Oncology

3.1.33. daratumumab - Orphan - EMEA-002152-PIP02-17

Janssen-Cilag International N.V.; Mature T-cell and Natural Killer-cell Neoplasms, Mature B-cell Neoplasms

Day 30 discussion

Action: For discussion

Oncology

3.1.34. fully human monoclonal antibody (mAb) directed against the human PD-1 receptor - EMEA-002007-PIP02-17

Treatment of Solid Tumours / Treatment of newly diagnosed diffuse intrinsic pontine gliomas (DIPG) and recurrent high-grade gliomas (HGG)

Day 30 discussion

Action: For discussion

Oncology

3.1.35. Talacotuzumab - EMEA-002158-PIP01-17

Acute myeloid leukaemia / Talacotuzumab, in combination with anti-cancer therapy is indicated for the treatment of pediatric patients, 28 days to 18 years of age with acute myeloid leukaemia

Day 30 discussion

Action: For discussion

Oncology / Haematology-Hemostaseology

3.1.36. Burosumab - EMEA-001659-PIP02-16

Tumor-induced osteomalacia

Day 30 discussion

Action: For discussion

Other

3.1.37. Fluticasone propionate - EMEA-002140-PIP01-17

Treatment of asthma (mild, moderate, and severe) / Prophylactic management in children who require prophylactic medication, including patients not controlled on currently available prophylactic medication

Day 30 discussion

Action: For discussion

Pneumology - Allergology

3.1.38. Salmeterol xinafoate / Fluticasone propionate - EMEA-002177-PIP01-17

Treatment of asthma (mild, moderate and severe) / Regular treatment of asthma where use of a combination product (long-acting β2 agonist and inhaled corticosteroid) is appropriate: • patients not adequately controlled with inhaled corticosteroids and “as needed” inhaled short-acting β2 agonist or • patients already adequately controlled on both inhaled corticosteroid and long-acting β2 agonist

Day 30 discussion

Action: For discussion

Pneumology - Allergology

3.1.39. Vilanterol trifenatate / Umeclidinium bromide / Fluticasone furoate - EMEA-002153-PIP01-17

ICD-10 J45.5x severe persistent asthma

Day 30 discussion

Action: For discussion

Pneumology - Allergology

3.1.40. Litoxetine (as benzoate) - EMEA-002151-PIP01-17

Bladder and urethral symptoms / Treatment of Mixed Urinary Incontinence (women), Treatment of Urinary Incontinence post prostatectomy (men)

Day 30 discussion

Action: For discussion

Uro-nephrology

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. Ataluren - EMEA-C2-000115-PIP01-07-M08

PTC Therapeutics International Limited; Treatment of dystrophinopathy

Day 30 discussion

Action: For discussion

Neurology

3.2.2. nivolumab - EMEA-C1-001407-PIP02-15-M01

Bristol-Myers Squibb Pharma EEIG; Treatment of malignant neoplasms of lymphoid tissue

Day 30 discussion

Action: For discussion

Oncology

3.2.3. Split influenza virus, inactivated containing antigen equivalent to A/H3N2-like strain / Split influenza virus, inactivated containing antigen equivalent to B-like strain (B/Yamagata lineage) / Split influenza virus, inactivated containing antigen equivalent to B-like strain (B/Victoria lineage) / Split influenza virus, inactivated containing antigen equivalent to A/H1N1-like strain - EMEA-C-001254-PIP01-11-M02

Sanofi Pasteur SA; Prevention of influenza infection

Day 30 discussion

Action: For discussion

Vaccines

3.3. Discussions on Modification of an Agreed Paediatric Investigation Plan

3.3.1. Captopril - EMEA-001544-PIP01-13-M01

Proveca Limited; Heart failure / Treatment of heart failure in children aged 2 to 18 years

Day 30 discussion

Action: For discussion

Cardiovascular Diseases

3.3.2. Tilmanocept - EMEA-001255-PIP01-11-M02

Norgine BV; Visualisation of lymphatic drainage of solid malignant tumours for diagnostic purposes / Visualisation of lymphatic drainage of rhabdomyosarcoma and melanoma for diagnostic purposes

Day 30 discussion

Action: For discussion

Diagnostic / Oncology

3.3.3. exenatide - EMEA-000689-PIP01-09-M07

AstraZeneca AB; Non insulin dependent diabetes mellitus (treatment including thiazolidinediones), Non insulin dependent diabetes mellitus (excluding treatment with thiazolidinediones), Non insulin dependent diabetes mellitus - in combination with insulin (with or without oral antidiabetics) / Treatment of type 2 Diabetes Mellitus

Day 30 discussion

Action: For discussion

Endocrinology-Gynaecology-Fertility-Metabolism

3.3.4. Human Fibrinogen - EMEA-001208-PIP01-11-M03

Octapharma Pharmazeutika Produktionsges. m. b. H; Treatment of congenital fibrinogen deficiency

Day 30 discussion

Action: For discussion

Haematology-Hemostaseology

3.3.5. cobicistat / darunavir - EMEA-001280-PIP01-12-M01

Janssen-Cilag International NV; Treatment of HIV-1 infection / Treatment of HIV-1 infection in pediatric patients from 3 to less than 18 years

Day 30 discussion

Action: For discussion

Infectious Diseases

3.3.6. doravirine - EMEA-001676-PIP01-14-M02

Merck Sharp & Dohme (Europe), Inc.; Treatment of human immunodeficiency virus-1 (HIV-1) infection / Antiretroviral therapy, in combination with other antiretroviral agents, for the treatment of HIV-1 infection in children aged from birth to 18 years

Day 30 discussion

Action: For discussion

Infectious Diseases

3.3.7. elbasvir / grazoprevir - EMEA-001604-PIP01-13-M03

Merck Sharp & Dohme (Europe), Inc.; treatment of chronic hepatitis C infection / Treatment of chronic hepatitis C genotype 1 infection with the combination regimen of MK-5172 and MK-8742 in children and adolescents from 3 years to less than 18 years of age who are previously untreated or who have failed previous Peg-Interferon/Interferon therapy with ribavirin

Day 30 discussion

Action: For discussion

Infectious Diseases

3.3.8. Fidaxomicin - EMEA-000636-PIP01-09-M06

Astellas Pharma Europe B.V.; Treatment of enterocolitis caused by clostridium difficile / Treatment of Clostridium difficile infections (CDI) also known as C. difficile-associated diarrhoea (CDAD)

Day 30 discussion

Action: For discussion

Infectious Diseases

3.3.9. tenofovir alafenamide / emtricitabine / cobicistat / darunavir - EMEA-001825-PIP01-15-M01

Janssen-Cilag International NV; Treatment of human immunodeficiency virus type-1 (HIV-1) infection / Treatment of HIV-1 infection in paediatric subjects weighing 25 kg or more above 6 years of age

Day 30 discussion

Action: For discussion

Infectious Diseases

3.3.10. Tenofovir disoproxil (as fumarate) - EMEA-000533-PIP01-08-M07

Gilead Sciences International Ltd; Treatment of human immunodeficiency virus (HIV-1) infection, Treatment of chronic viral hepatitis B / For treatment of chronic hepatitis B in paediatric patients from 2 years of age with compensated liver disease., In combination with other antiretroviral medicinal products for the treatment of HIV-1 infection in antiretroviral treatment experienced paediatric patients.

Day 30 discussion

Action: For discussion

Infectious Diseases

3.3.11. Tenofovir disoproxil fumarate / lamivudine / doravirine - EMEA-001695-PIP01-14-M02

Merck Sharp & Dohme (Europe), Inc.; Treatment of human immunodeficiency virus-1 (HIV-1) infection / Antiretroviral combination therapy, for the treatment of HIV-1 infection in adults and children aged 2 to 18 years

Day 30 discussion

Action: For discussion

Infectious Diseases

3.3.12. Brivaracetam - Orphan - EMEA-000332-PIP01-08-M12

UCB Pharma S.A.; Treatment of paediatric epilepsy syndromes, Treatment of epilepsy with partial onset seizures, Treatment of neonatal seizures / Treatment of neonatal seizures with adjunctive administration of brivaracetam, Treatment of paediatric patients with partial onset seizures, treatment of refractory paediatric epilepsy syndromes with adjunctive administration of brivaracetam

Day 30 discussion

Action: For discussion

Neurology

3.3.13. Daclizumab - EMEA-001349-PIP01-12-M02

Biogen Idec Ltd; Multiple Sclerosis / Treatment of relapsing remitting forms of multiple sclerosis

Day 30 discussion

Action: For discussion

Neurology

3.3.14. 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 discussion

Action: For discussion

Oncology

3.3.15. Dabrafenib (dabrafenib mesilate) - EMEA-001147-PIP01-11-M05

Novartis Europharm Limited; Treatment of solid malignant tumours (excluding melanoma), Treatment of melanoma / Treatment of paediatric patients with solid malignant tumours containing BRAF V600 mutations, Treatment of adolescent patients with melanoma with a BRAF V600 mutation

Day 30 discussion

Action: For discussion

Oncology

3.3.16. Poly(oxy-1,2-ethanediyl),alpha-hydro-omega-methoxy-133 ester with granulocyte colony-stimulating factor [methionyl,133-[O-[2-(acetylamino]-6-O-[N-[N-carboxyglycyl)amino]-alpha neuraminosyl]-2-deoxy-alpha-D-galactopyranosyl]-L-threonine]] (human) - EMEA-001019-PIP01-10-M04

UAB "Sicor Biotech"; Treatment of chemotherapy-induced neutropenia, Prevention of chemotherapy-induced febrile neutropenia / Treatment of neutropenia and reduction in the incidence of febrile neutropenia in patients treated with chemotherapy for malignancy

Day 30 discussion

Action: For discussion

Oncology

3.3.17. Sunitinib malate - EMEA-000342-PIP01-08-M06

Pfizer Limited; CD10 code C49.4 malignant neoplasms of connective and soft tissue of abdomen - gastro-intestinal stromal tumours (GIST) / Treatment of gastro-intestinal stromal tumour in paediatric patients aged 6 to less than 18

Day 30 discussion

Action: For discussion

Oncology

3.3.18. Trametinib (trametinib dimethyl sulfoxide) - EMEA-001177-PIP01-11-M04

Novartis Europharm Limited; treatment of melanoma, Treatment of solid malignant tumours (excluding melanoma) / Treatment of paediatric patients with solid malignant tumours containing BRAF V600 mutations, Treatment of adolescent patients with melanoma with a BRAF V600 mutation

Day 30 discussion

Action: For discussion

Oncology

3.3.19. sildenafil - Orphan - EMEA-000671-PIP01-09-M08

Pfizer Limited; Treatment of Pulmonary Arterial Hypertension (PAH)

Day 30 discussion

Action: For discussion

Other

3.3.20. lurasidone hydrochloride - EMEA-001230-PIP01-11-M03

Sunovion Pharmaceuticals Ltd.; schizophrenia

Day 30 discussion

Action: For discussion

Psychiatry

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 15 August 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

6.1.1. Antibody drug conjugate comprised of a humanized anti-HER2 antibody attached by a peptide linker to a novel topoisomerase I inhibitor - EMEA-10-2017

Daiichi Sankyo, Inc.; Class of Her- / Epidermal growth factor-receptor antibody medicinal products for treatment of breast malignant neoplasms/ Treatment of patients with human epidermal growth factor receptor (HER)2-positive metastatic breast cancer that is resistant or refractory to trastuzumab emtansine (T-DM1)

Action: For adoption

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. Evolocumab - EMEA-001268-PIP01-12-M04

Amgen Europe B.V.; Treatment of elevated cholesterol

Action: For discussion

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

Joint CHMP/PDCO session

Action: For discussion

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. Revision of the Guidelines on the clinical investigation and core SmPC of recombinant and human plasma-derived factor VIII products

Action: For discussion

9.3.4. Guideline on the clinical evaluation of medicinal products indicated for the prophylaxis or treatment of respiratory syncytial virus (RSV) disease

Action: For discussion

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

PDCO member: Maria Fernandez Cortizo

Action: For discussion

9.3.6. Draft Agenda of the PCWP/HCPWP joint meeting to be held on 27-28 June 2017

Action: Document tabled for information

9.4. Cooperation within the EU regulatory network

None

9.5. Cooperation with International Regulators

9.5.1. Gaucher disease - A strategic collaborative approach from EMA and FDA

PDCO member: Sylvie Benchetrit

Action: For adoption

9.5.2. Report on the EMA/FDA/Health Canada workshop on paediatric pulmonary arterial hypertension (PAH)' held on 12 June 2017 at EMA

Action: For information

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. Strategic Review and Learning Meeting (SRLM) to be held in Estonia on 4-6 October 2017

PDCO member: Irja Lutsar

Action: For discussion

10. Any other business

10.1.1. Paediatric applications to PDCO members: proposal for simplification

PDCO Chair: Dirk Mentzer

Action: For discussion

11. Breakout sessions

11.1.1. Paediatric oncology

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

11.1.2. Neonatology

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

11.1.3. Inventory

Action: For discussion on Friday, room 3A

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/