19 July 2017
EMA/406917/2017 Rev.
Inspections, Human Medicines Pharmacovigilance and Committees Division

Paediatric Committee (PDCO)

Draft agenda for the meeting on 18 – 21 July 2017

Chair: Dirk Mentzer – Vice-Chair: Koenraad Norga

18 July 2017, 14:00 - 19:00, room 3A

19 July 2017, 08:30 - 19:00, room 3A

20 July 2017, 08:30 - 19:00, room 3A

21 July 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 18 – 21 July 2017. See July 2017 PDCO minutes (to be published post August 2017 PDCO meeting)

1.2. Adoption of agenda

PDCO agenda for 18 – 21 July 2017

1.3. Adoption of the minutes

PDCO minutes for 20 – 23 June 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. 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 60 opinion

Action: For adoption

Oncology

2.1.2. Human normal immunoglobulin - EMEA-002084-PIP01-16

Primary Immunodeficiency Diseases

Day 90 discussion

Action: For discussion

Immunology-Rheumatology-Transplantation

2.1.3. Human Normal Immunoglobulin for Intravenous Administration (IVIg) - EMEA-002092-PIP01-16

Treatment of primary immunodeficiency (PID), Treatment of idiopathic thrombocytopenic purpura (ITP) / Primary immunodeficiency syndromes with impaired antibody production, Primary immune thrombocytopenia (ITP), in patients at high risk of bleeding or prior to surgery to correct the platelet count

Day 90 discussion

Action: For discussion

Immunology-Rheumatology-Transplantation

2.1.4. 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 60 opinion

Action: For adoption

Pneumology - Allergology

2.1.5. 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 60 opinion

Action: For adoption

Pneumology - Allergology

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

Birken AG; Treatment of epidermolysis bullosa

Day 120 opinion

Action: For adoption; Oral Explanation Meeting to be held on 19 July 2017, 14:00-15:00

Dermatology

2.1.7. 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 120 opinion

Action: For adoption

Gastroenterology-Hepatology

2.1.8. Atacicept - EMEA-002004-PIP01-16

Treatment of systemic lupus erythematosus

Day 120 opinion

Action: For adoption

Immunology-Rheumatology-Transplantation

2.1.9. Lefamulin - EMEA-002075-PIP01-16

Treatment of community-acquired pneumonia

Day 120 opinion

Action: For adoption

Infectious Diseases

2.1.10. 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 120 opinion

Action: For adoption

Neurology

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

Newron Pharmaceuticals SpA; Treatment of Rett syndrome

Day 120 opinion

Action: For adoption

Neurology

2.1.12. 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 120 opinion

Action: For adoption

Oncology

2.1.13. 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 120 opinion

Action: For adoption

Oncology

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

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

Day 60 opinion

Action: For adoption

Oncology

2.1.15. Burosumab - EMEA-001659-PIP02-16

Tumor-induced osteomalacia

Day 60 opinion

Action: For adoption

Other

2.1.16. 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 60 opinion

Action: For adoption

Uro-nephrology

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. vigabatrin - EMEA-C-000717-PIP02-13-M02

ORPHELIA Pharma SA; Treatment of epilepsy

Day 30 opinion

Action: For discussion

Neurology

2.3. Opinions on Modification of an Agreed Paediatric Investigation Plan

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

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

Day 60 opinion

Action: For adoption; Oral Explanation Meeting to be held on 20 July 2017, 15:00-16:00

Cardiovascular Diseases

2.3.2. 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 60 opinion

Action: For adoption

Diagnostic / Oncology

2.3.3. 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 60 opinion

Action: For adoption

Endocrinology-Gynaecology-Fertility-Metabolism

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

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

Day 60 opinion

Action: For adoption

Haematology-Hemostaseology

2.3.5. 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 60 opinion

Action: For adoption

Infectious Diseases

[1]

2.3.6. 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 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 60 opinion

Action: For adoption

Infectious Diseases

2.3.7. 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 60 opinion

Action: For adoption

Infectious Diseases

2.3.8. 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 60 opinion

Action: For adoption

[1] Procedure removed

Infectious Diseases

2.3.9. Tenofovir disoproxil (as fumarate) - EMEA-000533-PIP01-08-M07[2]

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 60 opinion

Action: For adoption

Infectious Diseases

2.3.10. 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 60 opinion

Action: For adoption

Neurology

2.3.11. 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 60 opinion

Action: For adoption

Oncology

2.3.12. 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

[2] Procedure added

Day 60 opinion

Action: For adoption

Oncology

2.3.13. 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 60 opinion

Action: For adoption

Oncology

2.3.14. 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 60 opinion

Action: For adoption; Oral Explanation Meeting to be held on 20 July 2017, 09:00-10:00

Oncology

2.3.15. 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 60 opinion

Action: For adoption

Oncology

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

Pfizer Limited; Treatment of Pulmonary Arterial Hypertension (PAH)

Day 60 opinion

Action: For adoption

Other

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

Sunovion Pharmaceuticals Ltd.; schizophrenia

Day 60 opinion

Action: For adoption

Psychiatry

2.4. Opinions on Re-examinations

2.4.1. CYSTEAMINE HYDROCHLORIDE - Orphan - EMEA-000322-PIP01-08-M05

ORPHAN EUROPE SARL; Cystinosis/ Treatment of corneal cystine crystal deposits in cystinosis

Day 30 opinion

Action: For adoption

Ophthalmology

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. Recombinant human monoclonal antibody to GM-CSF - EMEA-001882-PIP02-16

Treatment of chronic idiopathic arthritis (including rheumatoid arthritis, psoriatic arthritis, ankylosing spondylarthritis and juvenile idiopathic arthritis) / Treatment of active systemic juvenile idiopathic arthritis (sJIA) in patients 1 year of age and older, Treatment of active polyarticular juvenile idiopathic arthritis (pJIA) in patients 2 years of age and older

Day 90 discussion

Action: For discussion

Immunology-Rheumatology-Transplantation

3.1.2. tazobactam / ceftolozane - EMEA-001142-PIP02-16

Treatment of abdominal and gastrointestinal infections, Treatment of urinary tract infections, Treatment of pneumonia / Treatment of nosocomial pneumonia, Treatment of

complicated intra-abdominal infections (cIAI).

Day 90 discussion

Action: For discussion

Infectious Diseases

3.1.3. EMEA-002057-PIP01-16

Treatment of ischemic stroke to improve recovery

Day 90 discussion

Action: For discussion

Neurology

3.1.4. Pyridopyrimidione SMN2 Splicing Modifier - EMEA-002070-PIP01-16

Treatment of spinal muscular atrophy

Day 90 discussion

Action: For discussion

Neurology

3.1.5. EMEA-002072-PIP01-16

Treatment of select unresectable or metastatic solid tumours with epacadostat in combination with pembrolizumab in paediatric patients between the ages of 6 months and 18 years of age/ Select unresectable or metastatic solid tumours in paediatric patients >6 months and < 18 years

Day 90 discussion

Action: For discussion

Oncology

3.1.6. Autologous CD4+ and CD8+ T cells Expressing a CD19-Specific Chimeric Antigen Receptor - EMEA-001995-PIP01-16

Treatment of B-lymphoblastic leukemia/lymphoma, Treatment of mature B-cell neoplasms / Treatment of pediatric patients with relapsed or refractory CD19+ B-cell acute lymphoblastic leukemia, Treatment of pediatric patients with relapsed or refractory CD19+ B-NHL, including diffuse large B-cell lymphoma, Burkitt lymphoma, and primary mediastinal large B-cell lymphoma

Day 90 discussion

Action: For discussion

Oncology

3.1.7. Enasidenib - Orphan - EMEA-001798-PIP02-16

Celgene Europe Ltd; Treatment of Acute Myeloid Leukaemia / Treatment of patients aged 2 to 21 years old with relapsed or refractory IDH2- mutated AML after at least 2 prior induction attempts.

Day 90 discussion

Action: For discussion

Oncology

3.1.8. Entospletinib - EMEA-002058-PIP01-16

Treatment of Acute myeloid leukemia

Day 90 discussion

Action: For discussion

Oncology

3.1.9. Angiotensin II - EMEA-001912-PIP02-16

Catecholamine-resistant hypotension associated with distributive shock

Day 90 discussion

Action: For discussion

Other

3.1.10. EMEA-002082-PIP01-16

Treatment of cystic fibrosis / indicated to improve lung function and reduce pulmonary exacerbations for patients in all age groups with cystic fibrosis in conjunction with standard therapies.

Day 90 discussion

Action: For discussion

Pneumology - Allergology

3.1.11. tralokinumab - EMEA-001900-PIP02-17

Treatment of Atopic Dermatitis

Day 60 discussion

Action: For discussion

Dermatology

3.1.12. EMEA-002162-PIP01-17

Type 2 diabetes mellitus

Day 60 discussion

Action: For discussion

Endocrinology-Gynaecology-Fertility-Metabolism

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

Shire Pharmaceuticals Ireland Limited; Treatment of Progressive Familial Intrahepatic Cholestasis

Day 60 discussion

Action: For discussion

Gastroenterology-Hepatology

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

Irritable bowel syndrome (IBS)

Day 60 discussion

Action: For discussion

Gastroenterology-Hepatology

3.1.15. Hydroxycarbamide - EMEA-002156-PIP01-17

Sickle Cell Syndrome

Day 60 discussion

Action: For discussion

Haematology-Hemostaseology

3.1.16. Risankizumab - EMEA-001776-PIP02-17

Chronic Idiopathic Arthritis

Day 60 discussion

Action: For discussion

Immunology-Rheumatology-Transplantation

3.1.17. 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). / Primary Immunodeficiency Syndromes with failure of antibody production.

Day 60 discussion

Action: For discussion

Immunology-Rheumatology-Transplantation / Haematology-Hemostaseology

3.1.18. EMEA-002080-PIP01-16

Treatment of influenza

Day 60 discussion

Action: For discussion

Infectious Diseases

3.1.19. 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 60 discussion

Action: For discussion

Infectious Diseases

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

Intestinal malabsorption in preterm infants

Day 60 discussion

Action: For discussion

Neonatology - Paediatric Intensive Care / Gastroenterology-Hepatology

3.1.21. 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 60 discussion

Action: For discussion

Neonatology - Paediatric Intensive Care / Gastroenterology-Hepatology

3.1.22. 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 60 discussion

Action: For discussion

Oncology

3.1.23. 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 60 discussion

Action: For discussion

Oncology

3.1.24. Carotuximab - Orphan - EMEA-002138-PIP01-17

TRACON Pharma Limited--Patricia Bitar; Treatment of angiosarcoma

Day 60 discussion

Action: For discussion

Oncology

3.1.25. 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 60 discussion

Action: For discussion

Oncology

3.1.26. 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 60 discussion

Action: For discussion

Oncology / Haematology-Hemostaseology

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

ICD-10 J45.5x severe persistent asthma

Day 60 discussion

Action: For discussion

Pneumology - Allergology

3.1.28. Amlodipine / Perindopril arginine / Bisoprolol fumarate - EMEA-002173-PIP01-17

Treatment of vascular hypertensive disorders, Treatment of ischaemic coronary artery disorders

Day 30 discussion

Action: For discussion

Cardiovascular Diseases

3.1.29. amlodipine besylate / hydrochlorothiazide / candesartan cilexetil - EMEA-002174-PIP01-17

Treatment of essential hypertension

Day 30 discussion

Action: For discussion

Cardiovascular Diseases

3.1.30. Fluoromisonidazole (18F) - EMEA-001977-PIP04-17

Visualisation of tissue hypoxia in solid tumours for diagnostic purposes / Gliomas, Renal Cell Carcinoma, Sarcomas

Day 30 discussion

Action: For discussion

Diagnostic / Oncology

3.1.31. EMEA-002109-PIP01-16

K75.8 Other specified inflammatory liver diseases (non-alcoholic steatohepatitis [NASH]) /

Treatment of Non-Alcoholic Steatohepatitis (NASH) with mild to severe fibrosis (F1-F4) in paediatric subjects, 8 to < 18 years of age

Day 30 discussion

Action: For discussion

Gastroenterology-Hepatology

3.1.32. Crizanlizumab - Orphan - EMEA-002141-PIP01-17

Novartis Europharm Limited; Treatment of sickle cell disease

Day 30 discussion

Action: For discussion

Haematology-Hemostaseology

3.1.33. Filgotinib - EMEA-001619-PIP04-17

Treatment of chronic idiopathic arthritis (including rheumatoid arthritis, ankylosing spondylarthritis, psoriatic arthritis, and juvenile idiopathic arthritis) / Treatment of juvenile idiopathic arthritis

Day 30 discussion

Action: For discussion

Immunology-Rheumatology-Transplantation

3.1.34. Influenza virus surface antigens (haemagglutinin) of strain B (Yamagata lineage) / Influenza virus surface antigens (haemagglutinin) of strain A (H3N2) / Influenza virus surface antigens (haemagglutinin) of strain A (H1N1) / Influenza virus surface antigens (haemagglutinin) of strain B (Victoria lineage) - EMEA-002027-PIP02-17

Prevention of Influenza infection

Day 30 discussion

Action: For discussion

Infectious Diseases

3.1.35. EMEA-002184-PIP01-17

Treatment of excessive daytime sleepiness / Treatment of excessive daytime sleepiness in narcolepsy patients

Day 30 discussion

Action: For discussion

Neurology

3.1.36. D-Sorbitol / Naltrexone HCl / (RS)-Bacoflen - Orphan - EMEA-002164-PIP01-17

Pharnext SA; Charcot-Marie-Tooth disease Type 1A / Treatment of Charcot-Marie-Tooth Type 1A in symptomatic paediatric patients

Day 30 discussion

Action: For discussion

Neurology

3.1.37. Survival Motor Neuron Gene by Self-Complementary Adeno Associated Virus Serotype 9 (AAV9) - Orphan - EMEA-002168-PIP01-17

AveXis EU Limited; Spinal Muscular Atrophy

Day 30 discussion

Action: For discussion

Neurology

3.1.38. 16-base single-stranded PNA oligonucleotide linked to a 7 aminoacid peptide C214H290N114O57 - Orphan - EMEA-002119-PIP01-17

BIOGENERA SPA; Treatment of Neuroblastoma (NB) with MYCN expression/amplification

Day 30 discussion

Action: For discussion

Oncology

3.1.39. durvalumab - EMEA-002028-PIP01-16

Treatment of all conditions included in the category of malignant neoplasms (except central nervous system, haematopoietic and lymphoid tissue), Treatment of malignant neoplasms of haematopoietic and lymphoid tissue / Treatment of paediatric patients from birth to less than 18 years old with solid tumours, Treatment of paediatric patients from birth to less than 18 years old with haematological malignancies

Day 30 discussion

Action: For discussion

Oncology

3.1.40. Ixazomib - Orphan - EMEA-001410-PIP02-17

Takeda Pharm A/S; Treatment of Acute Lymphoblastic Leukaemia (ALL), Treatment of Multiple Myeloma (MM) / Treatment of adult patients with Newly Diagnosed Multiple Myeloma (NDMM), Treatment of paediatric patients diagnosed with relapsed precursor B-ALL or T-ALL

Day 30 discussion

Action: For discussion

Oncology

3.1.41. Lenalidomide - Orphan - EMEA-000371-PIP04-16

Celgene Europe Limited; treatment of mature b-cell neoplasms

Day 30 discussion

Action: For discussion

Oncology

3.1.42. palbociclib - EMEA-002146-PIP01-17

Treatment of rhabdomyosarcoma, Treatment of Ewing sarcoma / treatment of refractory or recurrent rhabdomyosarcoma, treatment of refractory or recurrent Ewing sarcoma

Day 30 discussion

Action: For discussion

Oncology

3.1.43. tremelimumab - EMEA-002029-PIP01-16

Treatment of all conditions included in the category of malignant neoplasms (except central nervous system, haematopoietic and lymphoid tissue), Treatment of malignant neoplasms of haematopoietic and lymphoid tissue / Treatment of paediatric patients from birth to less than 18 years old with solid tumours, Treatment of paediatric patients from birth to less than 18 years old with haematological malignancies

Day 30 discussion

Action: For discussion

Oncology

3.1.44. sodium thiosulfate - EMEA-002147-PIP02-17

Platinum-induced ototoxic hearing loss / Reducing ototoxicity in patients > 1 month and <18 years of age receiving cisplatin chemotherapy for standard risk hepatoblastoma

Day 30 discussion

Action: For discussion

Oncology / Oto-rhino-laryngology

3.1.45. Latanoprost / Netarsudil - EMEA-002175-PIP01-17

Treatment of Glaucoma

Day 30 discussion

Action: For discussion

Ophthalmology

3.1.46. (R) - azasetron (as besylate) - Orphan - EMEA-002165-PIP01-17

Sensorion SA; Otoxicity, poisoning due to cisplatin / Prevention of cisplatin-Induced otoxicity

Day 30 discussion

Action: For discussion

Oto-rhino-laryngology

3.1.47. benralizumab - EMEA-001214-PIP02-17

chronic rhinosinusitis with nasal polyposis

Day 30 discussion

Action: For discussion

Oto-rhino-laryngology

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. Human coagulation factor X - EMEA-C-000971-PIP01-10-M02

Bio Products Laboratory Ltd; Treatment of hereditary factor X deficiency

Day 30 discussion

Action: For discussion

Haematology-Hemostaseology

3.2.2. doravirine - EMEA-C1-001676-PIP01-14-M01

Merck Sharp & Dohme (Europe), Inc.; Treatment of human immunodeficiency virus type 1 (HIV-1) infection

Day 30 discussion

Action: For discussion

Infectious Diseases

3.2.3. lamivudine / tenofovir disoproxil funarate / doravirine - EMEA-C1-001695-PIP01-14-M01

Merck Sharp & Dohme (Europe), Inc.; Treatment of human immunodeficiency virus type 1 (HIV-1) infection

Day 30 discussion

Action: For discussion

Infectious Diseases

3.2.4. depatuxizumab mafodotin - EMEA-C1-001732-PIP02-15

AbbVie Ltd; Treatment of high-grade glioma

Day 30 discussion

Action: For discussion

Oncology

3.2.5. Burosumab - EMEA-C2-001659-PIP01-15-M02

Ultragenyx Pharmacetical Inc.; X-linked Hypophosphatemia

Day 30 discussion

Action: For discussion

Other

3.2.6. Ivacaftor - EMEA-C1-001640-PIP01-14-M01

Vertex Pharmaceuticals (Europe) Ltd; Treatment of cystic fibrosis

Day 30 discussion

Action: For discussion

Other / Pneumology - Allergology

3.3. Discussions on Modification of an Agreed Paediatric Investigation Plan

3.3.1. Alirocumab - EMEA-001169-PIP01-11-M03

Sanofi-aventis Recherche & Developpement; Treatment of elevated cholesterol / Treatment of heterozygous and homozygous familial hypercholesterolemia

Day 30 discussion

Action: For discussion

Endocrinology-Gynaecology-Fertility-Metabolism

3.3.2. Vestronidase alfa - Orphan - EMEA-001540-PIP01-13-M02

Ultragenyx Germany GmbH; ICD-10: E76.2, Mucopolysaccharidosis type VII (MPS VII) / Treatment of Mucopolysaccharidosis type VII (MPS VII)

Day 30 discussion

Action: For discussion

Endocrinology-Gynaecology-Fertility-Metabolism

3.3.3. Eluxadoline - EMEA-001579-PIP01-13-M01

Allergan Limited; Irritable bowel syndrome with diarrhoea

Day 30 discussion

Action: For discussion

Gastroenterology-Hepatology

3.3.4. Naloxegol (as naloxegol oxalate) - EMEA-001146-PIP01-11-M03

Kyowa Kirin Pharmaceutical Development Limited; Treatment of opioid-induced constipation

Day 30 discussion

Action: For discussion

Gastroenterology-Hepatology

3.3.5. Eltrombopag (eltrombopag olamine) - EMEA-000170-PIP03-13-M03

Novartis Europharm Limited; Bone Marrow Depression and Hypoplastic Anaemia / Treatment of cytopenias in paediatric patients with severe aplastic anaemia who are not receiving hematopoietic stem cell transplant

Day 30 discussion

Action: For discussion

Haematology-Hemostaseology

3.3.6. Human thrombin / Human fibrinogen - EMEA-001340-PIP01-12-M03

Mallinckrodt Pharmaceuticals Ireland Ltd; Treatment of haemorrhage resulting from a surgical procedure / Supportive treatment where standard surgical techniques are insufficient for improvement of haemostasis.

Day 30 discussion

Action: For discussion

Haematology-Hemostaseology

3.3.7. roxadustat - EMEA-001557-PIP01-13-M01

Astellas Pharma Europe B.V.; treatment of anaemia due to chronic disorders

Day 30 discussion

Action: For discussion

Haematology-Hemostaseology

3.3.8. Tenofovir alafenamide (as fumarate) - EMEA-001584-PIP01-13-M02

Gilead Sciences International Ltd.; Treatment of chronic hepatitis B / treatment of chronic hepatitis B infection in paediatric patients aged 2 years and above

Day 30 discussion

Action: For discussion

Infectious Diseases

3.3.9. Tasimelteon - Orphan - EMEA-001531-PIP01-13-M03

Vanda Pharmaceuticals; ICD-10 G47.24 Circadian rhythm sleep disorder, free-running type (Non-24) / Non24-Hour Sleep-Wake Disorder (Non-24) in the totally blind

Day 30 discussion

Action: For discussion

Neurology

3.3.10. L-asparaginase encapsulated in erythrocytes - Orphan - EMEA-000341-PIP02-09-M03

ERYTECH pharma S.A.; Treatment of acute lymphoblastic leukaemia / Treatment of patients with Acute Lymphoblastic Leukaemia

Day 30 discussion

Action: For discussion

Oncology

3.3.11. mepolizumab - Orphan - EMEA-000069-PIP02-10-M08

GlaxoSmithKline Trading Services; Treatment of asthma / add-on treatment for severe refractory eosinophilic asthma

Day 30 discussion

Action: For discussion

Pneumology – Allergology

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 12 September 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

4.3.1. Nomination of PDCO member to attend the '2nd Human Challenge Trials in Vaccine Development' to be held in Maryland, US on 28-30 September 2017

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. Olaparib - EMEA-11-2017

AstraZeneca AB; Treatment of breast carcinoma/monotherapy treatment for patients with metastatic gBRCAm HER2 negative breast cancer who are suitable for single agent chemotherapy when hormonal therapy is considered inappropriate

Action: For adoption

6.1.2. Olaparib - EMEA-12-2017

AstraZeneca AB; Treatment of adenocarcinoma of the pancreas/monotherapy maintenance treatment for gBRCAm metastatic pancreatic cancer patients whose disease has not progressed on first line platinum based chemotherapy

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. Ponesimod - EMEA-000798-PIP01-09

Actelion Pharmaceuticals Ltd.; Multiple Sclerosis/ Relapsing remitting forms of multiple sclerosis

Proposed indication: Add-on treatment for patients with relapsing forms of multiple sclerosis that are active despite treatment with dimethyl fumarate

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. Minutes PCWP/HCPWP joint meeting held at EMA on 15 March 2017

Action: Document tabled for information

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

PDCO members: Maria Fernandez Cortizo, Irja Lutsar

Action: For discussion

9.3.5. Guideline on good pharmacovigilance practices (GVP), 'Product- or Population-Specific Considerations IV: Paediatric population'[3]

PDCO Chair: Dirk Mentzer

Action: For adoption

9.4. Cooperation within the EU regulatory network

9.4.1. European Network of Paediatric Research (Enpr) – at European Medicines Agency (Enpr-EMA): Presentation of the European Cystic Fibrosis Clinial trial Network

Action: For discussion

9.4.2. The 2017 Commission Report on the Paediatric Regulation

Action: For information

9.5. Cooperation with International Regulators

None

[3] Addition of the latest revision of the Chapter of the GVP Product- or Population-Specific Considerations IV: Paediatric population

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. Draft Agenda of the Strategic Review and Learning Meeting to be held in Estonia on 4-6 October 2017

Action: For information

10. Any other business

10.1. None

11. Breakout sessions

11.1.1. Paediatric oncology

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

11.1.2. Neonatology

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

11.1.3. Inventory

Action: For discussion on Thursday, 14:00 - 15:00

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/