11 December 2017
EMA/PDCO/766329/2017
Inspections, Human Medicines Pharmacovigilance and Committees Division

Paediatric Committee (PDCO)

Draft agenda for the meeting on 12-15 December 2017

Chair: Dirk Mentzer – Vice-Chair: Koenraad Norga

12 December 2017, 14:00- 17:00, room 3E

13 December 2017, 08:30- 19:00, room 3E

14 December 2017, 08:30- 19:00, room 3E

15 December 2017, 08:30- 13:00, room 3E

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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Table of contents

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 December 2017. See December 2017 PDCO minutes (to be published post January 2018 PDCO meeting).

1.2. Adoption of agenda

PDCO agenda for 12-15 December 2017.

1.3. Adoption of the minutes

PDCO minutes for 7-10 November 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. Chloroprocaine Hydrochloride - EMEA-000639-PIP03-16

Peripheral nerve block (local anesthesia by perineural injection)

Day 120 opinion

Action: For adoption

Anaesthesiology

2.1.2. Lucerastat - Orphan - EMEA-002095-PIP01-16

Idorsia Pharmaceuticals Deutschland GmbH; Treatment of Fabry disease

Day 120 opinion

Action: For adoption

Endocrinology-Gynaecology-Fertility-Metabolism

2.1.3. - 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 120 opinion

Action: For adoption

Gastroenterology-Hepatology

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

Shire Pharmaceuticals Ireland Limited; Treatment of Progressive Familial Intrahepatic Cholestasis

Day 120 opinion

Action: For adoption

Gastroenterology-Hepatology

2.1.5. Glutamine (Levoglutamide) - Orphan - EMEA-001996-PIP02-16

Emmaus Medical Europe Ltd.; Sickle cell disease / Glutamine (Levoglutamide) is indicated for the prevention of sickle cell crises in adults and children older than 5 years suffering from Sickle Cell Disease.

Day 120 opinion

Action: For adoption

Haematology-Hemostaseology

2.1.6. Upadacitinib - EMEA-001741-PIP02-16

Treatment of Ulcerative Colitis

Day 120 opinion

Action: For adoption

Immunology-Rheumatology-Transplantation

2.1.7. 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

Day 120 opinion

Action: For adoption

Infectious Diseases

2.1.8. Gilteritinib (as fumarate) - EMEA-002064-PIP01-16

Treatment of acute myeloid leukemia / Treatment of FLT3/ITD positive acute myeloid leukemia

Day 120 opinion

Action: For adoption

Oncology / Haematology-Hemostaseology

2.1.9. 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 120 opinion

Action: For adoption

Vaccines

2.1.10. Rosuvastatin calcium / Acetylsalicylic acid - EMEA-002239-PIP01-17

Prevention of cardiovascular events

Day 60 opinion

Action: For adoption

Cardiovascular Diseases

2.1.11. Fluorocholine (18F) - EMEA-002129-PIP02-17

Visualisation of choline metabolism in malignant neoplasms

Day 60 opinion

Action: For adoption

Diagnostic

2.1.12. Tucatinib - EMEA-002242-PIP01-17

Treatment of breast malignant neoplasms

Day 60 opinion

Action: For adoption

Oncology

2.1.13. Recombinant human epidermal growth factor - EMEA-002258-PIP01-17

Diabetic foot ulcer

Day 60 opinion

Action: For adoption

Other / Endocrinology-Gynaecology-Fertility-Metabolism

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. Clostridium Botulinum neurotoxin type A (150 kD), free from complexing proteins - EMEA-C-001039-PIP01-10-M02

Merz Pharmaceuticals GmbH; Treatment of muscle spasticity

Day 60 opinion

Action: For adoption

Neurology

2.2.2. Clostridium Botulinum neurotoxin type A (150 kD), free of complexing proteins - EMEA-C1-001039-PIP02-12-M02

Merz Pharmaceuticals GmbH; Treatment of sialorrhea

Day 60 letter

Action: For adoption

Neurology

2.2.3. Dasatinib (as monohydrate) - EMEA-C-000567-PIP01-09-M04

Bristol-Myers Squibb Pharma EEIG; Treatment of Philadelphia chromosome (BCR-ABL translocation)-positive chronic myeloid leukaemia

Day 1 opinion

Action: For adoption

Oncology

2.3. Opinions on Modification of an Agreed Paediatric Investigation Plan

2.3.1. Regadenoson - EMEA-000410-PIP01-08-M02

Rapidscan Pharma Solutions EU Limited; Myocardial perfusion disturbances / Diagnostic evaluation of myocardial perfusion disturbances

Day 60 opinion

Action: For adoption

Cardiovascular Diseases

2.3.2. Gadolinium,[α3,α6,α9-tris[3-[[2-hydroxy-1-(hydroxymethyl)ethyl]amino]-3-oxopropyl]-3,6,9,15-tetraazabicyclo[9.3.1]pentadeca-1(15),11,13-triene-3,6,9-triacetato(3-)-κN3,κN6,κN9,κN15,κO3,κO6,κO9] - EMEA-001949-PIP01-16-M01

GUERBET; Detection and visualization of areas with disruption of the blood brain barrier and/or abnormal vascularity for the central nervous system (CNS), or of any type of diseases from different body regions (soft tissues, bone and internal body structures/organs) for diagnostic purposes.

Day 60 opinion

Action: For adoption

Diagnostic

2.3.3. Empagliflozin - EMEA-000828-PIP01-09-M06

Boehringer Ingelheim International GmbH; Treatment of type 2 diabetes mellitus

Day 60 opinion

Action: For adoption

Endocrinology-Gynaecology-Fertility-Metabolism

2.3.4. Linagliptin (as base) - EMEA-000498-PIP01-08-M07

Boehringer Ingelheim International GmbH; Type 2 Diabetes Mellitus

Day 60 opinion

Action: For adoption

Endocrinology-Gynaecology-Fertility-Metabolism

2.3.5. Sitagliptin phosphate - EMEA-000470-PIP01-08-M10

Merck Sharp and Dohme (Europe), Inc.; Treatment of type 2 diabetes mellitus

Day 60 opinion

Action: For adoption

Endocrinology-Gynaecology-Fertility-Metabolism

2.3.6. Baricitinib - EMEA-001220-PIP01-11-M02

Eli Lilly and Company Limited; Treatment of chronic idiopathic arthritis (including rheumatoid arthritis, ankylosing spondylarthritis, psoriatic arthritis and juvenile idiopathic arthritis) / Treatment of juvenile idiopathic arthritis, Treatment of JIA-associated uveitis

Day 60 opinion

Action: For adoption

Immunology-Rheumatology-Transplantation

2.3.7. Human normal immunoglobulin - EMEA-001797-PIP01-15-M01

Octapharma Pharmazeutika Produktionsges.m.b.H; Primary Immunodeficiency Diseases

Day 60 opinion

Action: For adoption

Immunology-Rheumatology-Transplantation

2.3.8. Ixekizumab - EMEA-001050-PIP01-10-M03

Eli Lilly & Company Limited; Plaque psoriasis, Treatment of chronic idiopathic arthritis (including rheumatoid arthritis, psoriatic arthritis, ankylosing spondylarthritis and juvenile idiopathic arthritis) / Children with juvenile idiopathic arthritis subtypes of enthesitis-related arthritis (including JoAS) and juvenile psoriatic arthritis., Treatment of severe chronic plaque psoriasis in paediatric patients from the age of 6 years who are not adequately controlled by topical therapies.

Day 60 opinion

Action: For adoption

Immunology-Rheumatology-Transplantation

2.3.9. Tofacitinib - EMEA-000576-PIP01-09-M08

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

2.3.10. Ustekinumab - EMEA-000311-PIP03-11-M03

Janssen-Cilag International NV; Treatment of chronic idiopathic arthritis (including rheumatoid arthritis, spondyloarthritis, PsA and juvenile idiopathic arthritis [JIA])

Day 60 opinion

Action: For adoption

Immunology-Rheumatology-Transplantation

2.3.11. Ceftaroline fosamil - EMEA-000769-PIP01-09-M07

Pfizer Limited; Treatment of cSSTI (complicated skin and soft tissue infections) / Treatment of CAP (community-aquired pneumonia)

Day 60 opinion

Action: For adoption

Infectious Diseases

2.3.12. Cobicistat / atazanavir sulphate - EMEA-001465-PIP01-13-M02

Bristol-Myers Squibb Pharma EEIG; Treatment of HIV-1 infection / indicated in combination with other ARV medicinal products for the treatment of HIV-1 infected adults and children from 3 years of age without known mutations associated with resistance to atazanavir.

Day 60 opinion

Action: For adoption

Infectious Diseases

2.3.13. Posaconazole - EMEA-000468-PIP02-12-M04

Merck Sharp & Dohme (Europe), Inc.; Prevention of invasive fungal infections / For treatment of invasive fungal infections in the following paediatric patients: -Invasive aspergillosis in patients with disease that is refractroy to amphotericin B or itraconazole or in patients who are intolerant of these medicinal products, Treatment of invasive aspergillosis, -Patients receiving remission-induction chemotherapy for acute myelogenous leukemia (AML) or myelodysplastic syndromes (MDS) expected to result in prolonged neutropenia and who are at high risk of developing invasive fungal infections; - Hematopoietic stem cell transplant (HSCT) recipients who are undergoing high-dose immunosuppressive therapy for graft versus host disease and who are at high risk of developing invasive fungal infections.

Day 60 opinion

Action: For adoption

Infectious Diseases

2.3.14. Tedizolid phosphate - EMEA-001379-PIP01-12-M03

Merck Sharp & Dohme (Europe) Inc.; Treatment of acute bacterial skin and skin structure infections

Day 60 opinion

Action: For adoption

Infectious Diseases

2.3.15. Tenofovir alafenamide - EMEA-001584-PIP01-13-M03

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

Day 60 opinion

Action: For adoption

Infectious Diseases

2.3.16. Lacosamide - EMEA-000402-PIP02-11-M05

UCB Pharma S.A.; Treatment of Epilepsy - Partial-onset seizures [G40.0 - G40.1 - G40.2]

Day 60 opinion

Action: For adoption

Neurology

2.3.17. Midostaurin - Orphan - EMEA-000780-PIP01-09-M04

Novartis Europharm Ltd; C92.0 Acute myeloid leukaemia, C94.3 Mast cell leukaemia, C96.2 Malignant mastocytosis / Treatment of paediatric patients with FLT3 mutated AML, newly diagnosed

Day 60 opinion

Action: For adoption

Oncology

2.3.18. Pembrolizumab - EMEA-001474-PIP02-16-M01

Merck Sharp & Dohme (Europe), Inc.; Treatment of all conditions included in the category of malignant neoplasms (except nervous system, haematopoietic and lymphoid tissue)., Treatment of Hodgkin Lymphoma / Treatment of advanced, untreated or previously treated, malignant melanoma in children from 12 year old to less than 18 years of age. Treatment as monotherapy of a PD-L1 positive paediatric malignant solid tumor in children from 6 months to less than 18 years of age., •Treatment of classical Hodgkin lymphoma with incomplete early response to front-linechemotherapy in children

from 3 years to less than 18 years of age •Treatment of relapsed or refractory classical Hodgkin lymphoma in children from 5 years to lessthan 18 years of age

Day 60 opinion

Action: For adoption

Oncology

2.3.19. Burosumab; Human recombinant IgG1 monoclonal antibody to fibroblast growth factor 23 (FGF23); KRN23 - Orphan - EMEA-001659-PIP01-15-M03

Ultragenyx Pharmaceutical Inc.; X-linked Hypophosphatemia

Day 60 opinion

Action: For adoption

Other

2.3.20. Conestat alfa - EMEA-000367-PIP01-08-M07

Pharming Group N.V.; D84.1 Defects in the complement system C1 esterase inhibitor (C1-INH) deficiency / Treatment of acute attacks of angioedema associated with hereditary C1 esterase inhibitor deficiency

Day 60 opinion

Action: For adoption

Other

2.3.21. Ivacaftor / lumacaftor - EMEA-001582-PIP01-13-M07

Vertex Pharmaceuticals (Europe) Limited; Treatment of cystic fibrosis

Day 60 opinion

Action: For adoption

Other

2.3.22. Matrix applied characterised autologous cultured chondrocytes - EMEA-000979-PIP01-10-M02

Vericel Denmark ApS; repair of symptomatic, full-thickness cartilage defects of the knee

Day 60 opinion

Action: For adoption

Other

2.3.23. Birch pollen extract (Betula verrucosa) - EMEA-001879-PIP01-15-M01

ALK Abelló A/S; J30.1 Allergic rhinitis due to pollen / Treatment of tree pollen allergic rhinitis and/or conjunctivitis

Day 60 opinion

Action: For adoption

Pneumology - Allergology

2.3.24. Reslizumab - EMEA-001202-PIP02-13-M02

Teva Pharmaceuticals Europe; Treatment of asthma / indicated as add- on treatment in adult patients with severe eosinophilic asthma

Day 60 opinion

Action: For adoption

Pneumology - Allergology

2.3.25. N-[(1,3-dicyclohexyl-6-hydroxy-2,4-dioxo-1,2,3,4-tetrahydro-5-pyrimidinyl)carbonyl]glycine - EMEA-001452-PIP01-13-M01

GlaxoSmithKline R & D; Treatment of anaemia associated with chronic renal disease

Day 60 opinion

Action: For adoption

Uro-nephrology / Haematology-Hemostaseology

2.3.26. Outer Membrane Vesicles (OMV) from Neisseria Meningitidis serogroup B strain NZ98/254 measured as amount of total protein containing the PorA P1.4 / Recombinant Neisseria Meningitidis serogroup B fHbp fusion protein / Recombinant Neisseria Meningitidis serogroup B NadA protein / Recombinant Neisseria Meningitidis serogroup B NHBA fusion protein - EMEA-000139-PIP01-07-M02

GSK Vaccines S.r.l.; Prevention of meningitis

Day 60 opinion

Action: For adoption

Vaccines

2.4. Opinions on Re-examinations

None

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. Tralokinumab - EMEA-001900-PIP02-17

Treatment of Atopic Dermatitis

Day 90 discussion

Action: For discussion

Dermatology

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

Irritable bowel syndrome (IBS)

Day 90 discussion

Action: For discussion

Gastroenterology-Hepatology

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

Novartis Europharm Limited; Treatment of sickle cell disease / Prevention of vaso-occlusive crises in patients with sickle cell disease

Day 90 discussion

Action: For discussion

Haematology-Hemostaseology

3.1.4. Plasminogen (human) - Orphan - EMEA-002044-PIP01-16

Prometic BioTherapeutics Ltd; Plasminogen deficiency

Day 90 discussion

Action: For discussion

Haematology-Hemostaseology

3.1.5. - EMEA-001741-PIP03-16

Treatment of Crohn's Disease

Day 90 discussion

Action: For discussion

Immunology-Rheumatology-Transplantation

3.1.6. Anifrolumab - EMEA-001435-PIP02-16

Lupus nephritis, Systemic lupus erythematosis

Day 90 discussion

Action: For discussion

Immunology-Rheumatology-Transplantation

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

Treatment of intestinal malabsorption in preterm infants

Day 90 discussion

Action: For discussion

Neonatology - Paediatric Intensive Care / Gastroenterology-Hepatology

3.1.8. Adeno-Associated Viral vector serotype rh.10 carrying the human N-sulfoglucosamine sulfohydrolase cDNA - Orphan - EMEA-002122-PIP02-17

LYSOGENE; Mucopolysaccharidosis type IIIA

Day 90 discussion

Action: For discussion

Neurology

3.1.9. 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 90 discussion

Action: For discussion

Neurology

3.1.10. 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 90 discussion

Action: For discussion

Oncology

3.1.11. 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 90 discussion

Action: For discussion

Oncology

3.1.12. 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 90 discussion

Action: For discussion

Oncology

3.1.13. 17α,21-dihydroxy-16α-methyl-pregna-1,4,9(11)-triene-3,20-dione - Orphan - EMEA-001794-PIP02-16

ReveraGen BioPharma Ltd; Treatment of duchenne muscular dystrophy

Day 90 discussion

Action: For discussion

Other

3.1.14. Tanezumab - EMEA-001635-PIP03-17

Treatment of chronic pain

Day 90 discussion

Action: For discussion

Pain

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

ICD-10 J45.5x severe persistent asthma

Day 90 discussion

Action: For discussion

Pneumology - Allergology

3.1.16. Baricitinib - EMEA-001220-PIP03-16

Treatment of atopic dermatitis / Treatment of patients with moderate to severe atopic dermatitis

Day 60 discussion

Action: For discussion

Dermatology

3.1.17. Bis-choline tetrathiomolybdate - Orphan - EMEA-002232-PIP01-17

Wilson Therapeutics AB; Treatment of Wilson disease

Day 60 discussion

Action: For discussion

Endocrinology-Gynaecology-Fertility-Metabolism

3.1.18. Ethinyl estradiol / Dienogest - EMEA-002229-PIP01-17

Oral contraception

Day 60 discussion

Action: For discussion

Endocrinology-Gynaecology-Fertility-Metabolism

3.1.19. Semaglutide - EMEA-001441-PIP03-17

Treatment of obesity

Day 60 discussion

Action: For discussion

Endocrinology-Gynaecology-Fertility-Metabolism

3.1.20. - Orphan - EMEA-002233-PIP01-17

Zealand Pharma A/S; Treatment of hypoglycaemia

Day 60 discussion

Action: For discussion

Endocrinology-Gynaecology-Fertility-Metabolism

3.1.21. Ustekinumab - EMEA-000311-PIP05-17

Treatment of Ulcerative Colitis

Day 60 discussion

Action: For discussion

Gastroenterology-Hepatology

3.1.22. (6aR,10aR)-1-Hydroxy-6,6-dimethyl-3-(2-methyl-2-octanyl)-6a,7,10,10a-tetrahydro-6H-benzo[c]chromene-9-carboxylic acid - Orphan - EMEA-002069-PIP02-17

Corbus Pharmaceuticals Holdings Inc; Treatment of systemic sclerosis

Day 60 discussion

Action: For discussion

Immunology-Rheumatology-Transplantation

3.1.23. Riociguat - Orphan - EMEA-000718-PIP03-17

Bayer AG; Treatment of Systemic Sclerosis / Treatment of Diffuse Cutaneous Systemic Sclerosis (dcSSc)

Day 60 discussion

Action: For discussion

Immunology-Rheumatology-Transplantation

3.1.24. The whole range of unmanipulated autologous mononuclear cells derived from human umbilical cord blood (Hau-UCB-mnc) - Orphan - EMEA-001799-PIP02-17

BrainRepair UG (haftungsbeschränkt); Periventriculaleukomalacia (PVL) ICD-10-CM P91.2

Day 60 discussion

Action: For discussion

Neonatology - Paediatric Intensive Care

3.1.25. Human donor hematopoietic stem and progenitor cells (HSPC) that have been treated ex vivo with Tat-MYC fusion protein - Orphan - EMEA-002185-PIP02-17

Taiga Biotechnologies, Inc.; Severe Combined Immunodeficiency

Day 60 discussion

Action: For discussion

Other / Immunology-Rheumatology-Transplantation

3.1.26. Purified Rabies virus - EMEA-002234-PIP01-17

Prevention of rabies disease, treatment of exposure to rabies virus

Day 60 discussion

Action: For discussion

Vaccines

3.1.27. Candesartan cilexetil / Amlodipine besylate - EMEA-002248-PIP01-17

Treatment of essential hypertension (ICD9: 401, ICD10: I10)

Day 30 discussion

Action: For discussion

Cardiovascular Diseases

3.1.28. Ezetimibe / Rosuvastatin - EMEA-002257-PIP01-17

Treatment of hypercholesterolemia / The combination of Ezetimibe and Rosuvastatin is indicated for the treatment of hypercholesterolemia as substitution therapy in adult patients adequately controlled with the individual substances given concurrently at the same dose level as in the fixed dose combination, but as separate products.

Day 30 discussion

Action: For discussion

Cardiovascular Diseases

3.1.29. Treprostinil sodium - Orphan - EMEA-002254-PIP01-17

SciPharm Sàrl; Treatment of (inoperable) chronic thromboembolic pulmonary hypertension (CTEPH)

Day 30 discussion

Action: For discussion

Cardiovascular Diseases

3.1.30. Birch bark extract - Orphan - EMEA-001299-PIP03-17

Amryt Research Limited; Treatment of epidermolysis bullosa

Day 30 discussion

Action: For discussion

Dermatology

3.1.31. a genetically modified Lactococcus lactis - EMEA-002237-PIP01-17

Treatment of Type 1 diabetes mellitus

Day 30 discussion

Action: For discussion

Endocrinology-Gynaecology-Fertility-Metabolism

3.1.32. Levothyroxine sodium - EMEA-002259-PIP01-17

Benign thyroid nodules, Goitre, Hypothyroidism

Day 30 discussion

Action: For discussion

Endocrinology-Gynaecology-Fertility-Metabolism

3.1.33. Metformin hydrochloride / dapagliflozin - EMEA-001151-PIP02-17

Type 2 diabetes (E11)

Day 30 discussion

Action: For discussion

Endocrinology-Gynaecology-Fertility-Metabolism

3.1.34. Metformin hydrochloride / saxagliptin / dapagliflozin - EMEA-002249-PIP01-17

Type 2 diabetes (E11)

Day 30 discussion

Action: For discussion

Endocrinology-Gynaecology-Fertility-Metabolism

3.1.35. Pemafibrate - EMEA-001573-PIP02-17

Treatment of hypertriglyceridaemia, Prevention of cardiovascular events in patients with elevated triglycerides levels

Day 30 discussion

Action: For discussion

Endocrinology-Gynaecology-Fertility-Metabolism / Cardiovascular Diseases

3.1.36. Venglustat - EMEA-002260-PIP01-17

ICD-10: G20; Disease of the nervous system; Extrapyramidal and movement disorders (G20-G26); Parkinson disease.

Day 30 discussion

Action: For discussion

Endocrinology-Gynaecology-Fertility-Metabolism / Neurology

3.1.37. - EMEA-001710-PIP03-17

Treatment of ulcerative colitis

Day 30 discussion

Action: For discussion

Gastroenterology-Hepatology

3.1.38. Risankizumab - EMEA-001776-PIP03-17

Crohn's Disease

Day 30 discussion

Action: For discussion

Gastroenterology-Hepatology

3.1.39. Risankizumab - EMEA-001776-PIP04-17

Ulcerative Colitis

Day 30 discussion

Action: For discussion

Gastroenterology-Hepatology

3.1.40. Autologous CD34+ haematopoietic stem cells transduced with lentiviral vector encoding the human βA-T87Q-globin gene - Orphan - EMEA-001665-PIP02-17

bluebird bio France; Sickle Cell Disease

Day 30 discussion

Action: For discussion

Haematology-Hemostaseology

3.1.41. Eptinezumab - EMEA-002243-PIP01-17

Prevention of migraine headaches

Day 30 discussion

Action: For discussion

Neurology

3.1.42. Entinostat Polymorph B - EMEA-002272-PIP01-17

Treatment of breast cancer

Day 30 discussion

Action: For discussion

Oncology

3.1.43. Niraparib - Orphan - EMEA-002268-PIP01-17

Janssen Research & Development; Treatment of prostate malignant neoplasms

Day 30 discussion

Action: For discussion

Oncology

3.1.44. Ruxolitinib phosphate - EMEA-000901-PIP04-17

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

Day 30 discussion

Action: For discussion

Oncology

3.1.45. T-cell bispecific antibody targeting carcinoembryonic antigen expressed on tumor cells and CD3 epsilon chain present on T-cells - EMEA-002252-PIP01-17

Treatment of all conditions included in the category of malignant neoplasms (except central nervous system tumours, haematopoietic and lymphoid tissue neoplasms

Day 30 discussion

Action: For discussion

Oncology

3.1.46. Veliparib - Orphan - EMEA-000499-PIP04-17

AbbVie Ltd; Treatment of lung carcinoma (SCLC and NSCLC)

Day 30 discussion

Action: For discussion

Oncology

3.1.47. Bilastine - EMEA-000347-PIP02-16

Treatment of allergic conjunctivitis

Day 30 discussion

Action: For discussion

Ophthalmology

3.1.48. Human Plasminogen - Orphan - EMEA-002253-PIP01-17

Kedrion S.p.A.; Treatment of Ligneous Conjunctivitis and prevention of pseudomembranes recurrence in patients affected by Ligneous Conjunctivitis

Day 30 discussion

Action: For discussion

Ophthalmology

3.1.49. Recombinant humanised monoclonal IgG2 lambda antibody against human sclerostin - Orphan - EMEA-002169-PIP01-17

Mereo Biopharma 3 Ltd; Treatment of osteogenesis imperfecta / Treatment of osteogenesis imperfecta, types 1, 3 and 4

Day 30 discussion

Action: For discussion

Other

3.1.50. Gabapentin / Trazodone hydrochloride - EMEA-002263-PIP01-17

Painful diabetic neuropathy

Day 30 discussion

Action: For discussion

Pain

3.1.51. Gefapixant citrate salt - EMEA-002267-PIP01-17

R05 - Symptoms and signs involving the circulatory and respiratory systems › Cough

Day 30 discussion

Action: For discussion

Pneumology - Allergology

3.1.52. Interferon beta-1a - Orphan - EMEA-002238-PIP01-17

Faron Pharmaceuticals Ltd; Treatment of Acute Respiratory Distress Syndrome

Day 30 discussion

Action: For discussion

Pneumology - Allergology

3.1.53. Ad26.RSV.preF - EMEA-002172-PIP02-17

Prevention of lower respiratory tract disease caused by respiratory syncytial virus

Day 30 discussion

Action: For discussion

Vaccines / Infectious Diseases

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. Sotagliflozin - EMEA-C1-001517-PIP02-14-M02

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

Day 30 discussion

Action: For discussion

Endocrinology-Gynaecology-Fertility-Metabolism

3.2.2. Testosterone - EMEA-C1-001529-PIP02-14

Acerus Pharmaceuticals SRL; Treatment of male hypogonadism

Day 30 discussion

Action: For discussion

Endocrinology-Gynaecology-Fertility-Metabolism

3.2.3. Tolvaptan - EMEA-C1-001231-PIP02-13-M05

Otsuka Pharmaceutical Europe Ltd.; Treatment of polycystic kidney disease

Day 30 discussion

Action: For discussion

Endocrinology-Gynaecology-Fertility-Metabolism

3.2.4. Avacopan - EMEA-C2-002023-PIP01-16-M02

ChemoCentryx, Ltd.; Treatment of ANCA-associated vasculitis

Day 30 discussion

Action: For discussion

Immunology-Rheumatology-Transplantation

3.2.5. Fenfluramine hydrochloride - EMEA-C1-001990-PIP01-16

Zogenix International Ltd; Treatment of Dravet syndrome

Day 30 discussion

Action: For discussion

Neurology

3.2.6. Fremanezumab - EMEA-C1-001877-PIP01-15-M01

Teva GmbH; Prevention of migraine headaches

Day 30 discussion

Action: For discussion

Neurology

3.2.7. Galcanezumab - EMEA-C3-001860-PIP03-16

Eli Lilly Nederland B.V.; Prevention of migraine headaches

Day 30 discussion

Action: For discussion

Neurology

3.2.8. Ozanimod - EMEA-C2-001710-PIP02-14-M02

Celgene Europe Limited; Treatment of Multiple Sclerosis

Day 30 discussion

Action: For discussion

Neurology

3.2.9. Avelumab - EMEA-C1-001849-PIP02-15-M01

Merck KGaA; Treatment of malignant neoplasms of lymphoid tissue

Day 30 discussion

Action: For discussion

Oncology

3.3. Discussions on Modification of an Agreed Paediatric Investigation Plan

3.3.1. Alirocumab - EMEA-001169-PIP01-11-M04

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. Osilodrostat - Orphan - EMEA-000315-PIP02-15-M01

Novartis Europharm Limited; Treatment of adrenal cortical hyperfunctions / Treatment of Cushing's disease in adolescents and children aged 6 years and older

Day 30 discussion

Action: For discussion

Endocrinology-Gynaecology-Fertility-Metabolism

3.3.3. Sodium zirconium cyclosilicate - EMEA-001539-PIP01-13-M03

AstraZeneca AB; Treatment of hyperkalemia

Day 30 discussion

Action: For discussion

Endocrinology-Gynaecology-Fertility-Metabolism

3.3.4. Autologous CD34+ haematopoietic stem cells transduced with lentiviral vector encoding the human betaA-T87Q-globin gene - Orphan - EMEA-001665-PIP01-14-M02

bluebird bio France; β-thalassaemia

Day 30 discussion

Action: For discussion

Haematology-Hemostaseology

3.3.5. Lonoctocog alfa - EMEA-001215-PIP01-11-M06

CSL Behring GmbH; Haemophilia A

Day 30 discussion

Action: For discussion

Haematology-Hemostaseology

3.3.6. Octocog alfa - EMEA-001064-PIP01-10-M03

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

Day 30 discussion

Action: For discussion

Haematology-Hemostaseology

3.3.7. Rolapitant - EMEA-001768-PIP02-15-M01

Tesaro UK Ltd; Chemotherapy-Induced Nausea and Vomiting (CINV) in Subjects Receiving Highly Emetogenic Chemotherapy (HEC) / Prevention of delayed nausea and vomiting associated with highly and moderately emetogenic cancer chemotherapy in adults given as part of combination therapy

Day 30 discussion

Action: For discussion

Haematology-Hemostaseology

3.3.8. Golimumab - EMEA-000265-PIP02-11-M02

Janssen Biologics B.V.; Treatment of ulcerative colitis

Day 30 discussion

Action: For discussion

Immunology-Rheumatology-Transplantation

3.3.9. Denosumab - EMEA-000145-PIP01-07-M09

Amgen Europe B.V.; Prevention of skeletal related events in patients with bone metastases, Treatment of hypercalcemia of malignancy, Treatment of chronic idiopathic artritis, Treatment of bone loss associated with sex hormone ablative therapy, Treatment

of giant cell tumour of bone / Treatment of giant cell tumour of bone in children (12-17 years old)

Day 30 discussion

Action: For discussion

Immunology-Rheumatology-Transplantation / Endocrinology-Gynaecology-Fertility-Metabolism / Oncology

3.3.10. Fidaxomicin - EMEA-000636-PIP01-09-M07

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

3.3.11. Piperaquine tetraphosphate / artenimol - EMEA-000153-PIP01-07-M05

Alfasigma SpA; Treatment of uncomplicated malaria caused by Plasmodium falciparum

Day 30 discussion

Action: For discussion

Infectious Diseases

3.3.12. Erenumab - EMEA-001664-PIP02-15-M02

Novartis Europharm Limited; Prevention of migraine headaches

Day 30 discussion

Action: For discussion

Neurology

3.3.13. Lacosamide - EMEA-000402-PIP03-17-M02

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 paediatric patients with idiopathic generalized epilepsy (IGE)(4 years to <18 years), Adjunctive therapy in the treatment of epileptic syndromes associated with generalized seizures in paediatric 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

3.3.14. Pyridopyrimidione SMN2 Splicing Modifier - EMEA-002070-PIP01-16-M01

Roche Registration Limited; Treatment of spinal muscular atrophy

Day 30 discussion

Action: For discussion

Neurology

3.3.15. Allogeneic T cells genetically modified with a retroviral vector encoding for a truncated low affinity nerve growth factor receptor (ΔLNGFR) and herpes simplex I virus thymidine kinase (HSV-Tk Mut2) - Orphan - EMEA-001370-PIP02-13-M01

MolMed S.p.A; Adjunctive treatment in haematopoietic cell transplantation

Day 30 discussion

Action: For discussion

Oncology

3.3.16. Binimetinib - EMEA-001454-PIP03-15-M01

PIERRE FABRE MEDICAMENT; Treatment of melanoma / Binimetinib in combination with encorafenib is indicated for the treatment of patients aged 12 years and older with unresectable or metastatic melanoma harbouring BRAF V600 mutations.

Day 30 discussion

Action: For discussion

Oncology

3.3.17. Encorafenib - EMEA-001588-PIP01-13-M01

PIERRE FABRE MEDICAMENT; Treatment of melanoma / Encorafenib in combination with binimetinib is indicated for the treatment of patients aged 12 years and older with unresectable or metastatic melanoma harbouring BRAF V600 mutations.

Day 30 discussion

Action: For discussion

Oncology

3.3.18. L-asparaginase encapsulated in erythrocytes - Orphan - EMEA-000341-PIP02-09-M05

ERYTECH pharma S.A.; Treatment of acute lymphoblastic leukaemia

Day 30 discussion

Action: For discussion

Oncology

3.3.19. Nivolumab - EMEA-001407-PIP01-12-M01

Bristol-Myers Squibb Pharma EEIG; Treatment of all conditions included in the category of malignant neoplasms (except nervous system, haematopoietic and lymphoid tissue) / Treatment of patients with unresectable or metastatic melanoma in the age group from 12 to less than 18 years old., Treatment of a paediatric malignant solid tumour in paediatric patients from 6 months to less than 18 years old.

Day 30 discussion

Action: For discussion

Oncology

3.3.20. Nivolumab - EMEA-001407-PIP02-15-M02

Bristol-Myers Squibb Pharma EEIG; Treatment of malignant neoplasms of lymphoid tissue, Treatment of malignant neoplasms of the central nervous system / Treatment of paediatric patients with relapsed or refractory Hodgkin lymphoma in the age group from 5 years to < 18 years., Treatment of paediatric patients with a relapsed or refractory non-Hodgkin lymphoma in the age group from 6 months to less than 18 years old., Treatment of paediatric patients from 6 months to less than 18 years of age with a recurrent or progressive high-grade glioma.

Day 30 discussion

Action: For discussion

Oncology

3.3.21. Pembrolizumab - EMEA-001474-PIP01-13-M01

Merck Sharp & Dohme (Europe), Inc.; Treatment of all conditions included in the category of malignant neoplasms (except nervous system, haematopoietic and lymphoid tissue). / Treatment of advanced, untreated or previously treated, malignant melanoma in children from 12 year old to less than 18 years of age. Treatment as monotherapy of a PD-L1 positive paediatric malignant solid tumor in children from 6 months to less than 18 years of age.

Day 30 discussion

Action: For discussion

Oncology

3.3.22. Selumetinib - EMEA-001585-PIP01-13-M02

AstraZeneca AB; Treatment of Thyroid Cancer, Treatment of Neurofibromatosis-Type 1 /

Selumetinib in combination with adjuvant radioactive iodine therapy is medicated for the treatment of adolescents newly diagnosed with differentiated thyroid cancer who are at high risk of primary treatment failure., Selumetinib is indicated for the treatment of inoperable NFl related plexiform neurofibroma in children and adolescents

Day 30 discussion

Action: For discussion

Oncology

3.3.23. Ivacaftor - Orphan - EMEA-000335-PIP01-08-M12

Vertex Pharmaceuticals (Europe) Limited; Treatment of Cystic Fibrosis

Day 30 discussion

Action: For discussion

Other

3.3.24. Dermatophagoides farinae / Dermatophagoides pteronyssinus - EMEA-001258-PIP01-11-M03

ALK-Abelló A/S; Treatment of allergic rhinitis, Treatment of asthma / indicated in house dust mite allergic asthma,

Day 30 discussion

Action: For discussion

Pneumology - Allergology

3.3.25. Ivacaftor - Orphan - EMEA-001640-PIP01-14-M04

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

Day 30 discussion

Action: For discussion

Pneumology - Allergology

3.3.26. Mometasone furoate / Indacaterol acetate (dose expressed as free base) - EMEA-001217-PIP01-11-M04

NOVARTIS EUROPHARM LTD.; Treatment of 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 27 February 2018 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. Trans-Capsaicin - EMEA-17-2017

Centrexion Therapeutics Corp; All classes of medicinal products for treatment of primary and secondary osteoarthrosis/Reduction in pain due to osteoarthritis

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. Tafamidis meglumine - EMEA-000884-PIP01-10

Pfizer Limited; neuropathic heredofamilial amyloidosis

Proposed indication: cardiomyopathy (due to wild-type or variant transthyretin)

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

Joint CHMP/PDCO session

Action: For discussion

9.2.2. Pharmacovigilance Risk Assessment Committee (PRAC)

Questions from PRAC to PDCO following a MS request for PRAC Advice

Action: For discussion

9.2.3. Coordination Group for Mutual Recognition and Decentralised Procedures - Human (CMDh)

CMDh Question to PDCO

PDCO member: Hugo Tavares

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. Guideline on the clinical evaluation of vaccines

Action: For discussion

9.3.4. Pilot phase on the Inventory of unmet needs

PDCO member: Karl-Heinz Huemer

Action: For information

9.4. Cooperation within the EU regulatory network

9.4.1. European Network of Paediatric Research (Enpr) - European Medicines Agency (EMA)

Action: For information

9.4.2. Allergen products

Action: For discussion

9.5. Cooperation with International Regulators

9.5.1. Food and Drug Administration (FDA)

Dates for 2018 FDA Pediatric Cluster Teleconference

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

9.7.1. Draft PDCO Work plan 2018

Action: For adoption

9.8. Planning and reporting

9.8.1. Business Pipeline Report - Forecast for 2017 - Update Q4/2017

Action: Tabled for information

10. Any other business

10.1. AOB topic

10.1.1. Preparedness of the system and capacity increase

Action: For information

11. Breakout sessions

None

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/