24 April 2018
EMA/257299/2018
Inspections, Human Medicines Pharmacovigilance and Committees Division

Paediatric Committee (PDCO)

Draft agenda for the meeting on 24-27 April 2018

Chair: Dirk Mentzer – Vice-Chair: Koenraad Norga

24 April 2018, 14:00- 19:00, room 3A

25 April 2018, 08:30- 19:00, room 3A

26 April 2018, 08:30- 19:00, room 3A

27 April 2018, 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 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 24-27 April 2018. See April 2018 PDCO minutes (to be published post April 2018 PDCO meeting).

1.2. Adoption of agenda

PDCO agenda for 24-27 April 2018.

1.3. Adoption of the minutes

PDCO minutes for 20-23 March 2018

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. Lasmiditan - EMEA-002166-PIP01-17

Migraine with and without aura

Day 120 opinion

Action: For adoption

Neurology

2.1.2. Setmelanotide - Orphan - EMEA-002209-PIP01-17

Rhythm Pharmaceuticals, Inc; Treatment of appetite and general nutrition disorders / Treatment of obesity and/or hyperphagia associated with genetic defects upstream of the MC4 receptor in the leptin-melanocortin pathway

Day 120 opinion

Action: For adoption

Nutrition

2.1.3. Daratumumab - Orphan - EMEA-002152-PIP01-17

Janssen-Cilag international N.V.; Lymphoid malignancies except mature B-cell neoplasms / Daratumumab in combination with standard chemotherapy is indicated for the treatment of paediatric patients from birth to 18 years with T-cell acute lymphoblastic leukemia and T-cell lymphoblastic lymphoma

Day 120 opinion

Action: For adoption

Oncology

2.1.4. Isatuximab - Orphan - EMEA-002205-PIP01-17

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

Day 120 opinion

Action: For adoption

Oncology

2.1.5. Trandolapril / verapamil - EMEA-002276-PIP01-17

Hypertension in adults

Day 60 opinion

Action: For adoption

Cardiovascular Diseases

2.1.6. Andecaliximab - EMEA-002304-PIP01-17

Treatment of gastric adenocarcinoma

Day 60 opinion

Action: For adoption

Oncology

2.1.7. Somapacitan - EMEA-001469-PIP02-17

Growth hormone deficiency, Short stature (ICD10 code: R6252). Treatment of paediatric patients with short stature born small for gestational age (SGA) with insufficient catch-up growth by age 2 to 4 years.

Day 60 adoption

Action: For adoption

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. Cobicistat / Darunavir - EMEA-C2-001280-PIP01-12-M01

Janssen-Cilag International NV; Treatment of HIV-1 infection

Day 60 letter

Action: For adoption

Infectious Diseases

2.2.2. Ozanimod - EMEA-C3-001710-PIP02-14-M02

Celgene Europe Limited; Treatment of Multiple Sclerosis

Day 60 letter

Action: For adoption

Neurology

2.2.3. Conestat Alfa - EMEA-C-000367-PIP01-08-M07

Pharming Group N.V.; Treatment of hereditary angioedema (HAE)

Day 60 opinion

Action: For adoption

Other

2.2.4. Eltrombopag - EMEA-C1-000170-PIP03-13-M03

Novartis Europharm Limited; Treatment of aplastic anaemia

Day 1 letter

Action: For adoption

Other

2.3. Opinions on Modification of an Agreed Paediatric Investigation Plan

2.3.1. Apixaban - EMEA-000183-PIP01-08-M06

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 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

2.3.2. Apixaban - EMEA-000183-PIP02-12-M02

Bristol-Myers Squibb / Pfizer EEIG; Treatment of venous thromboembolism

Day 60 opinion

Action: For adoption

Cardiovascular Diseases

2.3.3. Betrixaban - EMEA-001834-PIP02-16-M01

Portola Pharma UK Limited; Prevention of venous thromboembolism

Day 60 opinion

Action: For adoption

Cardiovascular Diseases

2.3.4. Apremilast - EMEA-000715-PIP03-11-M05

Celgene Europe Limited; Psoriasis in children

Day 60 opinion

Action: For adoption

Dermatology

2.3.5. Dupilumab - EMEA-001501-PIP01-13-M05

Regeneron Pharmaceuticals, Inc; atopic dermatitis

Day 60 opinion

Action: For adoption

Dermatology

2.3.6. Empagliflozin - EMEA-000828-PIP04-16-M01

Boehringer Ingelheim International GmbH; Treatment of type 1 diabetes mellitus

Day 60 opinion

Action: For adoption

Endocrinology-Gynaecology-Fertility-Metabolism

2.3.7. Human Fibrinogen - EMEA-001208-PIP01-11-M04

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

Day 60 opinion

Action: For adoption

Haematology-Hemostaseology

2.3.8. Baricitinib - EMEA-001220-PIP01-11-M03

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

2.3.9. Emapalumab - Orphan - EMEA-002031-PIP01-16-M01

Novimmune B.V; Treatment of Haemophagocytic Lymphohistiocytosis

Day 60 opinion

Action: For adoption

Immunology-Rheumatology-Transplantation

2.3.10. Ceftaroline fosamil - EMEA-000769-PIP01-09-M08

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

2.3.11. Ceftazidime / avibactam - EMEA-001313-PIP01-12-M07

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

Day 60 opinion

Action: For adoption

Infectious Diseases

2.3.12. Dasabuvir sodium monohydrate - EMEA-001439-PIP01-13-M02

Abbvie Ltd; Treatment of chronic hepatitis C / Treatment of children and adolescents from >= 3 years to less than 18 years of age with chronic HCV infection with compensated cirrhosis or without compensated cirrhosis in combination with ombitasvir, paritaprevir and ritonavir

Day 60 opinion

Action: For adoption

Infectious Diseases

2.3.13. - EMEA-001975-PIP01-16-M01

Janssen-Cilag International NV; Treatment of influenza

Day 60 opinion

Action: For adoption

Infectious Diseases

2.3.14. Lamivudine (3TC) / Dolutegravir (DTG) - EMEA-001940-PIP01-16-M01

ViiV Healthcare UK Limited; Treatment of human immunodeficiency virus (HIV-1) infection

Day 60 opinion

Action: For adoption

Infectious Diseases

2.3.15. Ritonavir / paritaprevir / ombitasvir - EMEA-001440-PIP01-13-M02

Abbvie Ltd; Chronic Hepatitis C (HCV) infection / Treatment of children and adolescents from >= 3 years to < 18 years of age with chronic HCV infection with compensated cirrhosis or without compensated cirrhosis in combination with other medicinal products

Day 60 opinion

Action: For adoption

Infectious Diseases

2.3.16. Sofosbuvir - EMEA-001276-PIP01-12-M02

Gilead Sciences International Ltd.; Treatment of chronic Hepatitis C in adolescents and children 3 years of age and older

Day 60 opinion

Action: For adoption

Infectious Diseases

2.3.17. Tenofovir Alafenamide / Emtricitabine - EMEA-001577-PIP02-14-M03

Gilead Sciences International Ltd.; Treatment of human immunodeficiency virus (HIV-1) infection.

Day 60 opinion

Action: For adoption

Infectious Diseases

2.3.18. Velpatasvir / Sofosbuvir - EMEA-001646-PIP01-14-M02

Gilead Sciences International Ltd.; Treatment of chronic Hepatitis C in adolescents and children 3 years of age and older

Day 60 opinion

Action: For adoption

Infectious Diseases

2.3.19. Fenfluramine hydrochloride - Orphan - EMEA-001990-PIP01-16-M01

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

Day 60 opinion

Action: For adoption

Neurology

2.3.20. Spheroids of human autologous matrix-associated chondrocytes - EMEA-001264-PIP01-12-M02

CO.DON AG; Treatment of symptomatic articular cartilage defects of the femoral condyle and the patella of the knee (International Cartilage Repair Society [ICRS] grade III or IV) with defect sizes up to 10 cm2

Day 60 opinion

Action: For adoption

Other

2.3.21. Formoterol fumarate dihydrate / Beclometasone dipropionate - EMEA-000548-PIP01-09-M08

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

Day 60 opinion

Action: For adoption

Pneumology - Allergology

2.3.22. Peanut flour - EMEA-001734-PIP01-14-M02

Aimmune Therapeutics Inc; Peanut Allergy / Peanut oral immunotherapy for the reduction in clinical reactivity to accidental exposure in peanut children and adults

Day 60 opinion

Action: For adoption

Pneumology - Allergology

2.3.23. Lurasidone hydrochloride - EMEA-001230-PIP01-11-M04

AZIENDE CHIMICHE RIUNITE ANGELINI FRANCESCO - A.C.R.A.F. S.p.A.; Schizophrenia

Day 60 opinion

Action: For adoption

Psychiatry

2.3.24. Etelcalcetide - EMEA-001554-PIP01-13-M02

Amgen Europe B.V.; Hyperparathyroid disorders / Hyperparathyroidism Secondary

Day 60 opinion

Action: For adoption

Uro-nephrology

2.3.25. Lubiprostone - EMEA-000245-PIP01-08-M05

Sucampo AG; Chronic idiopathic constipation

Day 30 adoption

Action: For adoption

Gastroenterology-Hepatology

2.4. Opinions on Re-examinations

None

2.5. Opinions on Review of Granted Waivers

2.5.1. Delafloxacin - EMEA-001080-PIP01-10

A.Menarini - IndustrieFarmaceutiche Riunite - s.r.I.; Treatment of local infections of skin and subcutaneous tissuesDay 30 adoption

Action: For adoption

Infectious Diseases

2.6. Finalisation and adoption of opinions

2.7. Partial Compliance Checks completed by EMA

For the following partial compliance checks, no need to refer them to PDCO Committee for discussion was identified by the PME coordinator and PDCO Rapporteur. The PDCO has been informed in writing

None

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. Bimekizumab - EMEA-002189-PIP01-17

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

Day 90 discussion

Action: For discussion

Dermatology

3.1.2. EMEA-002216-PIP01-17

Treatment of Atopic Dermatitis

Day 90 discussion

Action: For discussion

Dermatology

3.1.3. Dasiglucagon - Orphan - EMEA-002233-PIP01-17

Zealand Pharma A/S; Treatment of hypoglycaemia

Day 90 discussion

Action: For discussion

Endocrinology-Gynaecology-Fertility-Metabolism

3.1.4. Ustekinumab - EMEA-000311-PIP05-17

Treatment of Ulcerative Colitis

Day 90 discussion

Action: For discussion

Gastroenterology-Hepatology

3.1.5. Itacitinib - Orphan - EMEA-002178-PIP01-17

Incyte Biosciences UK Ltd.; Treatment of steroid naïve paediatric population with acute graft versus host disease after allogeneic hematopoietic stem cell transplantation

Day 90 discussion

Action: For discussion

Immunology-Rheumatology-Transplantation

3.1.6. Recombinant IgG degrading enzyme of Streptococcus pyogenes - Orphan - EMEA-002183-PIP01-17

Hansa Medical AB; Patients with chronic kidney disease in need of kidney transplantation / Prevention of graft rejection following solid organ transplantation

Day 90 discussion

Action: For discussion

Immunology-Rheumatology-Transplantation

3.1.7. The whole range of unmanipulated autologous mononuclear cells derived from human umbilical cord blood - Orphan - EMEA-001799-PIP02-17

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

Day 90 discussion

Action: For discussion

Neonatology - Paediatric Intensive Care

3.1.8. EMEA-002184-PIP01-17

Treatment of obstructive sleep apnoea, Treatment of narcolepsy, Treatment of excessive daytime sleepiness in narcolepsy patients

Day 90 discussion

Action: For discussion

Neurology

3.1.9. Palbociclib - EMEA-002146-PIP01-17

Treatment of refractory or recurrent Ewing sarcoma

Day 90 discussion

Action: For discussion

Oncology

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

Prevention of rabies disease, treatment of exposure to rabies virus

Day 90 discussion

Action: For discussion

Vaccines

3.1.11. Mavacamten - EMEA-002231-PIP01-17

Treatment of obstructive Hypertrophic Cardiomyopathy

Day 60 discussion

Action: For discussion

Cardiovascular Diseases

3.1.12. Brincidofovir - Orphan - EMEA-001904-PIP03-18

Chimerix UK Limited; Treatment of smallpox

Day 60 discussion

Action: For discussion

Infectious Diseases

3.1.13. Ibalizumab - EMEA-002311-PIP01-17

Treatment of human immunodeficiency virus (HIV-1) infection / Ibalizumab, a CD4 domain 2-directed HIV-1 inhibitor, in combination with other antiretroviral(s), treatment of children and adolescents (aged 6 to less than 18 years) infected with HIV-1 resistant to at least 1 agent in 3 different classes.

Day 60 discussion

Action: For discussion

Infectious Diseases

3.1.14. Pretomanid - Orphan - EMEA-002115-PIP01-17

Global Alliance for TB Drug Development; Treatment of multi-drug-resistant tuberculosis

Day 60 discussion

Action: For discussion

Infectious Diseases

3.1.15. Rezafungin acetate - EMEA-002319-PIP01-17

Treatment of invasive candidiasis

Day 60 discussion

Action: For discussion

Infectious Diseases

3.1.16. Tedizolid phosphate - EMEA-001379-PIP03-17

Treatment of Gram-positive bacterial pneumonia

Day 60 discussion

Action: For discussion

Infectious Diseases

3.1.17. Brigatinib - EMEA-002296-PIP01-17

Inflammatory Myofibroblastic Tumors (IMT), Non-small cell lung cancer (NSCLC), Anaplastic large cell lymphoma (ALCL) / Treatment of anaplastic lymphoma kinase (ALK) positive advanced non-small cell lung cancer (NSCLC), Treatment of paediatric patients ≥2 years of age with ALK+ unresectable or recurrent IMT, Treatment in combination with standard chemotherapy in paediatric patients ≥2 years of age with newly diagnosed ALK+ ALCL at high risk for recurrence.

Day 60 discussion

Action: For discussion

Oncology

3.1.18. Palovarotene - EMEA-001662-PIP03-17

Treatment of Multiple Osteochondromas (MO)

Day 60 discussion

Action: For discussion

Other

3.1.19. Meloxicam / Bupivacaine - EMEA-002246-PIP01-17

Acute Post Operative Pain

Day 60 discussion

Action: For discussion

Pain / Anaesthesiology

3.1.20. - EMEA-002297-PIP02-18

Treatment of Microvascular Coronary Artery Disease

Day 30 discussion

Action: For discussion

Cardiovascular Diseases

3.1.21. Allogeneic bone-marrow derived adherent, ex-vivo expanded multipotent adult progenitor cells product - EMEA-002317-PIP01-17

Acute ischaemic stroke

Day 30 discussion

Action: For discussion

Cardiovascular Diseases

3.1.22. Dapagliflozin - EMEA-000694-PIP03-17

I50 Heart Failure

Day 30 discussion

Action: For discussion

Cardiovascular Diseases

3.1.23. Moxonidine - EMEA-002275-PIP01-17

Treatment of Hypertension

Day 30 discussion

Action: For discussion

Cardiovascular Diseases

3.1.24. Trandolapril - EMEA-002274-PIP01-17

Mild or moderate hypertension

Day 30 discussion

Action: For discussion

Cardiovascular Diseases

3.1.25. Patidegib - EMEA-002322-PIP01-17

Treatment of basal cell carcinoma (BCC)

Day 30 discussion

Action: For discussion

Dermatology / Oncology

3.1.26. Evinacumab - EMEA-002298-PIP01-17

Treatment of elevated cholesterol / Treatment of homozygous familial hypercholesterolemia (HoFH)

Day 30 discussion

Action: For discussion

Endocrinology-Gynaecology-Fertility-Metabolism

3.1.27. Ianalumab - EMEA-002338-PIP01-18

Treatment of autoimmune hepatitis in patients aged 12 years to <18 years in whom steroids and/or azathioprine are contraindicated, are not tolerated, or do not provide an adequate response

Day 30 discussion

Action: For discussion

Gastroenterology-Hepatology

3.1.28. Abatacept - EMEA-000118-PIP04-17

Treatment of childhood-onset of Sjögren's Syndrome

Day 30 discussion

Action: For discussion

Immunology-Rheumatology-Transplantation

3.1.29. Liposomal ciclosporin A (L-CsA) - Orphan - EMEA-002344-PIP01-18

Breath Therapeutics GmbH; Treatment of Bronchiolitis obliterans Syndrome (BOS)

Day 30 discussion

Action: For discussion

Immunology-Rheumatology-Transplantation

3.1.30. Upadacitinib Hemihydrate - EMEA-001741-PIP05-17

Treatment of Vasculitides

Day 30 discussion

Action: For discussion

Immunology-Rheumatology-Transplantation

3.1.31. EMEA-002240-PIP02-17

Treatment of Urinary Tract Infections

Day 30 discussion

Action: For discussion

Infectious Diseases

3.1.32. Reltecimod - Orphan - EMEA-002325-PIP01-18

Atox Bio Ltd.; Treatment for necrotizing soft tissue infections

Day 30 discussion

Action: For discussion

Infectious Diseases

3.1.33. EMEA-001970-PIP02-17

Treatment of Clostridium difficile infection / indicated to reduce recurrence of Clostridium difficile infection (CDI) in paediatric patients who have received antibacterial drug treatment for recurrent CDI.

Day 30 discussion

Action: For discussion

Infectious Diseases

3.1.34. Isoflurane - EMEA-002320-PIP01-17

Sedation

Day 30 discussion

Action: For discussion

Neonatology - Paediatric Intensive Care

3.1.35. Fostamatinib - EMEA-001196-PIP02-17

Treatment of immune thrombocytopenia (idiopathic thrombocytopenic purpura)

Day 30 discussion

Action: For discussion

Other

3.1.36. Bupivacaine - EMEA-000877-PIP03-17

postsurgical analgesia

Day 30 discussion

Action: For discussion

Pain

3.1.37. Nitrous oxide - EMEA-002340-PIP01-18

anesthesia, analgesia / sedation / In analgesia / sedation in all conditions in which pain relief / sedation with rapid onset and rapid fall in effect is required (short-term surgical interventions, traumatology, burns, dentistry, otorhinolaryngology, childbirth), Under anesthesia, in combination with other anesthetics administered by inhalation or intravenously.

Day 30 discussion

Action: For discussion

Pain / Anaesthesiology

3.1.38. Calcifediol - EMEA-002093-PIP02-17

Treatment of secondary hyperparathyroidism (SHPT) in non-dialysis chronic kidney disease (ND-CKD) patients with low serum 25-hydroxyvitamin D levels

Day 30 discussion

Action: For discussion

Uro-nephrology

3.1.39. EMEA-002353-PIP01-18

Prevention of influenza infection

Day 30 discussion

Action: For discussion

Vaccines

3.1.40. Bilastine - EMEA-000347-PIP03-18

Treatment of urticaria / Treatment of allergic rhinoconjunctivitis

Day 30 discussion

Action: For discussion

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. Osilodrostat - EMEA-C1-000315-PIP02-15-M01

Novartis Europharm Limited; Treatment of adrenal cortical hyperfunctions

Day 30 discussion

Action: For discussion

Endocrinology-Gynaecology-Fertility-Metabolism

3.2.2. Fc- and CDR-modified humanised monoclonal antibody against C5 - EMEA-C2-002077-PIP01-16-M01

Alexion Europe SAS; Treatment of Paroxysmal Nocturnal Haemoglobinuria

Day 30 discussion

Action: For discussion

Haematology-Hemostaseology

3.2.3. Birch pollen extract (Betula verrucosa) - EMEA-C1-001879-PIP01-15-M01

ALK Abelló A/S; Treatment of allergic rhinitis / rhino-conjunctivitis

Day 30 discussion

Action: For discussion

Pneumology - Allergology

3.2.4. Angiotensin II - EMEA-C1-001912-PIP02-16-M01

La Jolla Pharmaceutical II B.V.; Treatment of hypotension associated with distributive or vasodilatory shockDay 30 discussion

Action: For discussion

Cardiovascular diseases

3.3. Discussions on Modification of an Agreed Paediatric Investigation Plan

3.3.1. Bempedoic acid - EMEA-001872-PIP01-15-M01

Esperion Therapeutics, Inc.; Treatment of elevated cholesterol / Treatment of heterozygous and homozygous familial hypercholesterolemia

Day 30 discussion

Action: For discussion

Cardiovascular Diseases

3.3.2. Regadenoson - EMEA-000410-PIP01-08-M03

GE Healthcare AS; Diagnostic evaluation of myocardial perfusion disturbances

Day 30 discussion

Action: For discussion

Cardiovascular Diseases

3.3.3. Ticagrelor - EMEA-000480-PIP01-08-M11

AstraZeneca AB; thromboembolic events (children), acute coronary syndrome, history of myocardial infarction / reduction in occurrence of vaso-occlusive crises in paediatric patients with sickle cell disease

Day 30 discussion

Action: For discussion

Cardiovascular Diseases / Haematology-Hemostaseology

3.3.4. Brodalumab - EMEA-001089-PIP02-13-M01

LEO Pharma A/S; Treatment of psoriasis

Day 30 discussion

Action: For discussion

Dermatology

3.3.5. Ligelizumab - EMEA-001811-PIP02-15-M02

Novartis Europharm Ltd.; Treatment of chronic spontaneous urticaria

Day 30 discussion

Action: For discussion

Dermatology

3.3.6. 2-hydroxypropyl-ß-cyclodextrin (HP-ß-CD) - Orphan - EMEA-001866-PIP01-15-M02

Mallinckrodt Pharmaceuticals Ireland Ltd; Treatment of progressive neurological manifestations in children and adolescent patients with Niemann-Pick disease, type C

Day 30 discussion

Action: For discussion

Endocrinology-Gynaecology-Fertility-Metabolism

3.3.7. Empagliflozin - EMEA-000828-PIP01-09-M07

Boehringer Ingelheim International GmbH; Treatment of type 2 diabetes mellitus

Day 30 discussion

Action: For discussion

Endocrinology-Gynaecology-Fertility-Metabolism

3.3.8. Glycerol phenylbutyrate - Orphan - EMEA-000297-PIP02-12-M02

Horizon Pharma Ireland Limited; E72.2 Urea cycle disorders / indicated for use as adjunctive therapy for chronic management of patients with urea cycle disorders (UCDs) including deficiencies of carbamoyl phosphate-synthase-I (CPS), ornithine carbamoyltransferase (OTC), argininosuccinate synthetase (ASS), argininosuccinate lyase (ASL), arginase I (ARG) and ornithine translocase deficiency hyperornithinaemia-hyperammonaemia homocitrullinuria syndrome (HHH) who cannot be managed by dietary protein restriction and/or amino acid supplementation alone.

Day 30 discussion

Action: For discussion

Endocrinology-Gynaecology-Fertility-Metabolism

3.3.9. Linagliptin - EMEA-000498-PIP01-08-M08

Boehringer Ingelheim International GmbH; Treatment of type 2 diabetes mellitus

Day 30 discussion

Action: For discussion

Endocrinology-Gynaecology-Fertility-Metabolism

3.3.10. Potassium chloride / Sodium chloride / Citric acid, anhydrous / Sodium citrate / Simeticone / Sodium sulphate, anhydrous / Macrogol 4000 - EMEA-001356-PIP02-12-M02

Alfasigma S.p.A.; any clinical procedures requiring a clean bowel e.g. bowel endoscopy or radiology.

Day 30 discussion

Action: For discussion

Gastroenterology-Hepatology

3.3.11. Vonicog alfa - Orphan - EMEA-001164-PIP01-11-M02

Baxalta Innovations GmbH; Von Willebrand Disease / Treatment and control of haemorrhage (spontaneous and surgical) and prevention of bleeding in surgery in paediatric patients (age of < 18 years) diagnosed with VWD when desmopressin (DDAVP) treatment alone is ineffective or not indicated.

Day 30 discussion

Action: For discussion

Haematology-Hemostaseology

3.3.12. Eculizumab - Orphan - EMEA-000876-PIP05-15-M03

Alexion Europe SAS; Treatment of Refractory Generalized Myasthenia Gravis

Day 30 discussion

Action: For discussion

Immunology-Rheumatology-Transplantation

3.3.13. Expanded donor-derived allogenic T cells transduced with the retroviral vector expressing the transgenes for inducible caspase9 and the truncated CD19 selectable marker - Orphan - EMEA-001869-PIP01-15-M01

Bellicum Pharma Ltd; Adjunctive treatment in haematopoietic stem cell transplantation

Day 30 discussion

Action: For discussion

Immunology-Rheumatology-Transplantation

3.3.14. Rimiducid - Orphan - EMEA-001870-PIP01-15-M01

Bellicum Pharma Ltd.; Treatment of graft versus host disease

Day 30 discussion

Action: For discussion

Immunology-Rheumatology-Transplantation

3.3.15. Tofacitinib - EMEA-000576-PIP01-09-M09

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

Day 30 discussion

Action: For discussion

Immunology-Rheumatology-Transplantation

3.3.16. Pibrentasvir / Glecaprevir - EMEA-001832-PIP01-15-M01

AbbVie Ltd; Treatment of Chronic Hepatitis C

Day 30 discussion

Action: For discussion

Infectious Diseases

3.3.17. Arimoclomol citrate - Orphan - EMEA-001748-PIP01-15-M01

Orphazyme A/S; Treatment of Niemann-Pick Disease, Type C

Day 30 discussion

Action: For discussion

Neurology

3.3.18. Domagrozumab - Orphan - EMEA-001763-PIP01-15-M02

Pfizer Ltd; Duchenne Muscular Dystrophy

Day 30 discussion

Action: For discussion

Neurology

3.3.19. Pitolisant - Orphan - EMEA-001176-PIP01-11-M03

BIOPROJET PHARMA; Narcolepsy with or without cataplexy

Day 30 discussion

Action: For discussion

Neurology

3.3.20. Tasimelteon - Orphan - EMEA-001531-PIP01-13-M04

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

3.3.21. Cobimetinib - EMEA-001425-PIP01-13-M03

Roche Registration Limited; Treatment of all conditions included in the category of malignant neoplasms (except haematopoietic and lymphoid tissue) with Ras, Raf or MEK pathway activation / Treatment of children with a paediatric solid malignant tumour with known or expected Ras, Raf or MEK pathway activation, at first relapse or refractory to initial treatment.

Day 30 discussion

Action: For discussion

Oncology

3.3.22. Larotrectinib - Orphan - EMEA-001971-PIP02-16-M01

Bayer AG; Treatment of all conditions included in the category of malignant neoplasms (except central nervous system tumours, haemtopoietic and lymphoid tissue neoplasms). / Treatment of paediatric patients from birth to less than 18 years of age with advanced solid tumours harbouring an NTRK fusion.

Day 30 discussion

Action: For discussion

Oncology

3.3.23. Sirolimus - Orphan - EMEA-001416-PIP01-12-M02

Santen Incorporated; Treatment of non-infectious uveitis affecting the posterior segment of the eye

Day 30 discussion

Action: For discussion

Ophthalmology

3.3.24. Autologous CD34+ enriched cell fraction that contains CD34+ cells transduced with lentiviral vector that encodes for the human ARSA cDNA sequence - Orphan - EMEA-001765-PIP02-15-M02

GlaxoSMithKline Trading Services Limited; For the treatment of Metachromatic leukodystrophy (MLD)

Day 30 discussion

Action: For discussion

Other

3.3.25. Calcium chloride / Aprotinin / Fibrinogen / Thrombin - EMEA-001079-PIP01-10-M04

Kedrion S.p.A.; T81.0 Haemorrhage and haematoma complicating a procedure / Treatment and prevention of haemorrhage resulting from a surgical procedure

Day 30 discussion

Action: For discussion

Other

3.3.26. Mexiletine hydrochloride - Orphan - EMEA-002012-PIP01-16-M01

Lupin (Europe) Ltd.; Symptomatic treatment of myotonic disorders

Day 30 discussion

Action: For discussion

Other

3.3.27. Gabapentin - EMEA-001310-PIP01-12-M03

PHARM Srl; Treatment of chronic pain in paediatric patients aged from 3 months to less than 18 years

Day 30 discussion

Action: For discussion

Pain

3.3.28. Tezepelumab - EMEA-001613-PIP01-14-M01

AstraZeneca AB; Treatment of asthma / Tezepelumab is indicated as add-on maintenance treatment of patients with severe asthma aged 5 years and older.

Day 30 discussion

Action: For discussion

Pneumology - Allergology

3.3.29. Potassium hydrogen carbonate / Potassium citrate monohydrate - EMEA-001535-PIP01-13-M01

Advicenne; Cystinuria (ICD 10: E72.0)

Day 30 discussion

Action: For discussion

Uro-nephrology

3.3.30. Sucroferric oxyhydroxide - EMEA-001061-PIP01-10-M03

Vifor Fresenius Medical Care Renal Pharma France; Hyperphosphataemia / Control of serum phosphorus levels in paediatric and adolescent subjects with chronic kidney disease (CKD)

Day 30 discussion

Action: For discussion

Uro-nephrology

3.3.31. Influenza virus surface antigens (haemagglutinin and neuraminidase) of the following strains: A/<Official Strain>(H1N1), A/<Official Strain>(H3N2), B/<Official Strain>Yamagata lineage, B/<Official Strain>Victoria lineage based on annual recommendations by WHO, CHMP (EU) and other regional or local authorities - EMEA-001782-PIP01-15-M03

Abbott Biologicals B.V.; Prevention of Influenza infection / Prophylaxis of influenza;

especially in those who run an increased risk of associated complications

Day 30 discussion

Action: For discussion

Vaccines

3.3.32. Recombinant Varicella Zoster Virus (VZV) glycoprotein E antigen - EMEA-001426-PIP01-13-M02

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

Day 30 discussion

Action: For discussion

Vaccines

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 26 June 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. Inhibitor of ADAMTS-5 – EMEA-03-2018

LES LABORATOIRES SERVIER; All classes of medicinal products for treatment of primary and secondary osteoarthrosis/ Treatment of mild to moderate osteoarthritis of the knee and hip to reduce the degradation of cartilage

Action: For adoption

6.1.2. Poly(oxy-1,2-ethanediyl), alpha-hydro-.omega.-hydroxy-,15,15'-diester with N-acetyl-L-isoleucyl-L-cysteinyl-L-valyl-1-methyl-L-tryptophyl-L-glutaminylL-.alpha.-aspartyl-L-tryptophylglycyl-L-alanyl-L-histidyl-L-arginyl-L-cysteinylL-threonyl-2-[2-(2-aminoethoxy)ethoxy]acetyl-N6-carboxy-L-lysinamide cyclic (2.fwdarw.12)-(disulfide); where two identical synthetic peptide domains are covalently linked to the ends of the polyethylene glycol chain - EMEA-04-2018

Apellis Pharmaceuticals Inc; All classes of medicinal products for treatment of age-related macular degeneration and diabetic macular oedema/ Treatment of geographic atrophy secondary to age related macular degeneration

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

None

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

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

CHMP/PDCO joint session

PDCO Member: Martina Rieg

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

9.4. 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. Bundesinstitut für Arzneimittel und Medizinprodukte (BfArM)

Request for PDCO advice (EMEA-000018-PIP01-07-M13)

PDCO member: Sabine Scherer

Action: For discussion

9.5. Cooperation with International Regulators

None

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

None

9.7. PDCO work plan

None

9.8. Planning and reporting

9.8.1. Business Pipeline Report - Forecast for 2018 - Update Q1/2018

Action: Tabled for information

10. Any other business

10.1.1. Training needs for PDCO members and alternates

Action: For discussion

10.1.2. Preparedness of the system and capacity increase

Action: For information

10.1.3. Collaborative papers of PDCO with Rome Foundation on Irritable Bowel Syndrome and Functional Constipation in children – POSTPONED TO MAY

PDCO member: Johannes Taminiau

Action: For information

10.1.4. Haemophilia registries workshop

Action: For information

10.1.5. Workshop on EMA stakeholder interaction on the development of medicinal products for chronic non-infectious liver diseases (PBC, PSC and NASH)

Action: For information

10.1.6. EC/EMA action plan to further improve the implementation of the Paediatric Regulation

Scope: Next steps for development of action plan

Action: For information

10.1.7. Involvement of young people into PDCO activities

Action: For information

11. Breakout sessions

11.1.1. Paediatric oncology

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

11.1.2. Neonatology

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

11.1.3. Inventory

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

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/