27 May 2019
EMA/PDCO/250257/2019
Inspections, Human Medicines Pharmacovigilance and Committees Division

Paediatric Committee (PDCO)

Draft agenda for the meeting on 27-29 May 2019

Chair: Dirk Mentzer – Vice-Chair: Koenraad Norga

27 May 2019, 08:30- 19:00, room 2D

28 May 2019, 08:30- 19:00, room 2D

29 May 2019, 08:30- 16:00, room 2D

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 on 27-29 May 2019. See 27-29 May 2019 PDCO minutes (to be published post 25-28 June 2019 PDCO meeting).

1.2. Adoption of agenda

PDCO agenda for 27-29 May 2019 meeting

1.3. Adoption of the minutes

PDCO minutes for 23-26 April 2019 meeting

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. Emricasan - EMEA-002457-PIP01-18

Non-alcoholic steatohepatitis (NASH) / Treatment of NASH with moderate to severe liver fibrosis (F2-F4) in patients aged 8 to less than 18 years old

Day 120 Opinion

Action: For adoption

Gastroenterology-Hepatology

2.1.2. Tropifexor - EMEA-002471-PIP01-18

Non-alcoholic steatohepatitis (NASH) / Treatment of NASH with moderate to severe liver fibrosis (F2/F3) in paediatric patients from 8 to less than 18 years of age

Day 120 Opinion

Action: For adoption

Gastroenterology-Hepatology

2.1.3. 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 120 Opinion

Action: For adoption

Haematology-Hemostaseology

2.1.4. Allogeneic Epstein-Barr virus specific cytotoxic T lymphocytes - Orphan - EMEA-002025-PIP02-16

Atara Biotherapeutics, Inc.; Treatment of Epstein-Barr virus associated post-transplant lymphoproliferative disorder / Treatment of solid organ transplant (SOT) patients with Epstein-Barr virus associated post transplant lymphoproliferative disease (EBV+ PTLD) who have failed prior therapy with rituximab /Treatment of allogeneic haematopoietic cell transplant (alloHCT) patients with Epstein-Barr virus associated post transplant lymphoproliferative cisease (EBV+ PTLD) who have failed prior therapy with rituximab

Day 120 Opinion

Action: For adoption

Oncology

2.1.5. EMEA-002310-PIP02-17

Treatment of C3 glomerulopathy

Day 120 Opinion

Action: For adoption

Uro-nephrology

2.1.6. Bisoprolol fumarate / ramipril - EMEA-002560-PIP01-19

Treatment of essential hypertension in adults / treatment of heart failure in adults

Day 60 Opinion

Action: For adoption

Cardiovascular Diseases

2.1.7. Ezetimibe / rosuvastatin calcium - EMEA-002541-PIP01-18

Elevated cholesterol

Day 60 Opinion

Action: For adoption

Cardiovascular Diseases

2.1.8. Heparin sodium - EMEA-002557-PIP01-19

Prevention of thromboembolic events

Day 60 Opinion

Action: For adoption

Cardiovascular Diseases

2.1.9. Ritonavir / darunavir - EMEA-002537-PIP01-18

Treatment of human immunodeficiency virus (HIV-1) infection

Day 60 Opinion

Action: For adoption

Infectious Diseases

2.1.10. EMEA-002318-PIP03-19

Treatment of malignant melanoma

Day 60 Opinion

Action: For adoption

Oncology

2.1.11. bemarituzumab (anti-FGFR2b humanised immunoglobulin G1 (IgG1) monoclonal antibody) - EMEA-002401-PIP01-18

Treatment of all conditions included in the category of malignant neoplasms (except central nervous system tumours, haematopoietic and lymphoid tissue neoplasms) overexpressing FGFR2b

Day 60 Opinion

Action: For adoption

Oncology

2.1.12. Momelotinib - Orphan - EMEA-001656-PIP02-19

Sierra Oncology Inc.; Treatment of Primary Myelofibrosis

Day 60 Opinion

Action: For adoption

Oncology

2.1.13. Moxetumomab pasudotox - Orphan - EMEA-002525-PIP01-18

AstraZeneca AB; Chronic lymphocytic leukaemias

Day 60 Opinion

Action: For adoption

Oncology

2.1.14. Bempedoic acid - EMEA-001872-PIP02-19

Treatment of mixed dyslipidaemia

Day 60 Opinion

Action: For adoption

Other / Cardiovascular Diseases

2.1.15. Ezetimibe / bempedoic acid - EMEA-002200-PIP02-19

Treatment of mixed dyslipidaemia

Day 60 Opinion

Action: For adoption

Other / Cardiovascular Diseases

2.2. Opinions on Compliance Check

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

2.2.1. Filgotinib - EMEA-C1-001619-PIP04-17-M01

Gilead Sciences International Ltd.; Treatment of chronic idiopathic arthritis (including rheumatoid arthritis, ankylosing spondylarthritis, psoriatic arthritis, and juvenile idiopathic arthritis)

Day 60 Letter

Action: For adoption

Immunology-Rheumatology-Transplantation

2.2.2. Lefamulin - EMEA-C1-002075-PIP01-16-M01

Nabriva Therapeutics AG; Treatment of community-acquired pneumonia

Day 60 letter

Action: For adoption

Infectious Diseases

2.3. Opinions on Modification of an Agreed Paediatric Investigation Plan

2.3.1. Baricitinib - EMEA-001220-PIP03-16-M01

Eli Lilly and Company Limited; Treatment of atopic dermatitis / Treatment of patients with moderate to severe atopic dermatitis

Day 60 Opinion

Action: For adoption

Dermatology

2.3.2. Terbinafine hydrochloride - EMEA-001259-PIP02-13-M02

Polichem, S.A.; Treatment of onychomycosis

Day 60 Opinion

Action: For adoption

Dermatology

2.3.3. Testosterone - EMEA-001529-PIP02-14-M02

Acerus Biopharma Inc.; Male hypogonadism / Treatment of male hypogonadism

Day 60 Opinion

Action: For adoption

Endocrinology-Gynaecology-Fertility-Metabolism

2.3.4. Human normal immunoglobulin for subcutaneous administration - EMEA-001853-PIP01-15-M02

Grifols Therapeutics LLC; Treatment for primary immunodeficiency

Day 60 Opinion

Action: For adoption

Immunology-Rheumatology-Transplantation

2.3.5. Tocilizumab - EMEA-000309-PIP04-17-M02

Roche Registration GmbH; Cytokine release syndrome / Treatment of chimeric antigen receptor (CAR) T cell-induced severe or life-threatening cytokine release syndrome (CRS) in adults and paediatric patients 2 years of age and older

Day 60 Opinion

Action: For adoption

Immunology-Rheumatology-Transplantation

2.3.6. Eravacycline - EMEA-001555-PIP01-13-M03

Tetraphase Pharmaceuticals, Inc.; Complicated intra-abdominal infection

Day 60 Opinion

Action: For adoption

Infectious Diseases

2.3.7. Posaconazole - EMEA-000468-PIP02-12-M05

Merck Sharp & Dohme (Europe), Inc.; Prevention of invasive fungal infections / Treatment of invasive fungal infections / Treatment of invasive fungal infections in the following paediatric patients: invasive aspergillosis in patients with disease that is refractory 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

2.3.8. Tedizolid phospate - EMEA-001379-PIP01-12-M04

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

Day 60 Opinion

Action: For adoption

Infectious Diseases

2.3.9. Balovaptan - EMEA-001918-PIP01-15-M02

Roche Registration GmbH; ICD1O F84: Treatment of autism spectrum disorder / Treatment of core social and communication deficits in people with autism spectrum disorder aged 2 years or older

Day 60 Opinion

Action: For adoption

Neurology

2.3.10. Eculizumab - Orphan - EMEA-000876-PIP03-14-M03

Alexion Europe SAS; Neuromyelitis optica spectrum disorders / Treatment of paediatric patients with relapsing neuromyelitis optica spectrum disorders

Day 60 Opinion

Action: For adoption

Neurology

2.3.11. Galcanezumab - EMEA-001860-PIP03-16-M03

Eli Lilly and Company Limited; Prevention of migraine headaches

Day 60 Opinion

Action: For adoption

Neurology

2.3.12. Humanised anti-interleukin-6 (IL-6) receptor (IL-6R) monoclonal antibody - Orphan - EMEA-001625-PIP01-14-M03

Roche Registration GmbH; Treatment of neuromyelitis optica

Day 60 Opinion

Action: For adoption

Neurology

2.3.13. Afatinib - EMEA-001596-PIP02-17-M01

Boehringer Ingelheim International GmbH; Treatment of all conditions included in the category of malignant neoplasms (except central nervous system, haematopoietic and lymphoid tissue neoplasms) / Treatment of malignant neoplasms of the central nervous system

Day 60 Opinion

Action: For adoption

Oncology

2.3.14. Durvalumab - EMEA-002028-PIP01-16-M01

AstraZeneca AB; Treatment of malignant neoplasms of haematopoietic and lymphoid tissue / Treatment of all conditions included in the category of malignant neoplasms (except central nervous system, haematopoietic and lyphoid tissue) / Treatment of paediatric patients from birth to less than 18 years old with a paediatric haematological malignancy / Treatment of paediatric patients from birth to less than 18 years old with a paediatric solid tumour

Day 60 Opinion

Action: For adoption

Oncology

2.3.15. Tremelimumab - EMEA-002029-PIP01-16-M01

AstraZeneca AB; Treatment of malignant neoplasms of haematopoietic and lymphoid tissue / Treatment of all conditions included in the category of malignant neoplasms (except central nervous system, haematopoietic and lyphoid tissue) / Treatment of paediatric patients from birth to less than 18 years old with a paediatric haematological malignancy / Treatment of paediatric patients from birth to less than 18 years old with a paediatric solid tumour

Day 60 Opinion

Action: For adoption

Oncology

2.3.16. Venetoclax - Orphan - EMEA-002018-PIP02-16-M02

AbbVie Ltd; Treatment of haematopoietic and lymphoid malignant neoplasms / Treatment of solid tumour malignant neoplasms / Treatment of a specific paediatric hematopoietic and lymphoid malignant neoplasm or of a solid malignant neoplasm in patients from 1 month to 18 years of age

Day 60 Opinion

Action: For adoption

Oncology / Haematology-Hemostaseology

2.3.17. Concentrate of proteolytic enzyme enriched in bromelain - Orphan - EMEA-000142-PIP02-09-M08

MediWound Germany GmbH; Treatment of burns of external body surfaces / Removal of eschar in deep partial and/or full thickness burns

Day 60 Opinion

Action: For adoption

Other

2.3.18. Lanadelumab - Orphan - EMEA-001864-PIP01-15-M04

Shire Pharmaceuticals Ireland Limited (an indirect wholly owned subsidiary of Shire plc); Hereditary angioedema / Treatment of hereditary angioedema

Day 60 Opinion

Action: For adoption

Other

2.3.19. Fentanyl hydrochloride - EMEA-001509-PIP01-13-M02

Incline Therapeutics Europe Ltd. (a wholly owned subsidiary of The Medicines Company); Treatment of acute pain

Day 60 Opinion

Action: For adoption

Pain

2.3.20. Ivacaftor / tezacaftor - Orphan - EMEA-001640-PIP01-14-M05

Vertex Pharmaceuticals (Europe) Ltd.; Cystic fibrosis / Treatment of cystic fibrosis

Day 60 Opinion

Action: For adoption

Pneumology - Allergology

2.3.21. Pneumococcal Polysaccharide Serotype 33F – Diphtheria CRM197 Conjugate / Pneumococcal Polysaccharide Serotype 23F – Diphtheria CRM197 Conjugate / Pneumococcal Polysaccharide Serotype 22F – Diphtheria CRM197 Conjugate / Pneumococcal Polysaccharide Serotype 19F – Diphtheria CRM197 Conjugate / Pneumococcal Polysaccharide Serotype 19A – Diphtheria CRM197 Conjugate / Pneumococcal Polysaccharide Serotype 18C – Diphtheria CRM197 Conjugate / Pneumococcal Polysaccharide Serotype 14 – Diphtheria CRM197 Conjugate / Pneumococcal Polysaccharide Serotype 9V – Diphtheria CRM197 Conjugate / Pneumococcal Polysaccharide Serotype 7F – Diphtheria CRM197 Conjugate / Pneumococcal Polysaccharide Serotype 6B – Diphtheria CRM197 Conjugate / Pneumococcal Polysaccharide Serotype 6A – Diphtheria CRM197 Conjugate / Pneumococcal Polysaccharide Serotype 5 – Diphtheria CRM197 Conjugate / Pneumococcal Polysaccharide Serotype 4 – Diphtheria CRM197 Conjugate / Pneumococcal Polysaccharide Serotype 3 – Diphtheria CRM197 Conjugate / Pneumococcal Polysaccharide Serotype 1 – Diphtheria CRM197 Conjugate - EMEA-002215-PIP01-17-M02

Merck Sharp & Dohme (Europe), Inc.; Prevention of disease caused by streptococcus pneumoniae / Active immunisation for the prevention of invasive disease, pneumonia and acute otitis media caused by streptococcus pneumoniae in infants, children and adolescents from 6 weeks to less than 18 years of age

Day 60 Opinion

Action: For adoption

Vaccines

2.3.22. Esketamine hydrochloride - EMEA-001428-PIP03-15-M01

Janssen-Cilag International NV; Major depressive disorder (MDD)

Day 60 Opinion

Action: For adoption

Psychiatry

2.4. Opinions on Re-examinations

2.4.1. Agomelatine - EMEA-001181-PIP01-11-M04

Les Laboratoires Servier; Treatment of major depressive episodes

Action: For adoption

Psychiatry

2.5. Opinions on Review of Granted Waivers

No items

2.6. Finalisation and adoption of Opinions

No items

2.7. Partial Compliance Checks completed by EMA

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

2.7.1. Mirikizumab - EMEA-C1-002208-PIP01-17

Eli Lilly and Company; Treatment of psoriasis

Day 1 letter

Action: For information

Dermatology / Gastroenterology-Hepatology

2.7.2. Isatuximab - EMEA-C1-002205-PIP01-17-M01

Sanofi-Aventis Recherche & Développement; Treatment of malignant neoplasms of the haematopoietic and lymphoid tissue

Day 1 letter

Action: For information

Oncology

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. EMEA-002329-PIP01-18

Treatment of dermatitis and eczema

Day 90 discussion

Action: For discussion

Dermatology

3.1.2. EMEA-002350-PIP01-18

Treatment of psoriasis / Treatment of moderate to severe plaque psoriasis in paediatric patients 6 years of age and older

Day 90 discussion

Action: For discussion

Dermatology

3.1.3. EMEA-002464-PIP01-18

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

Day 90 discussion

Action: For discussion

Dermatology

3.1.4. Oxalobacter formigenes strain HC-1 - Orphan - EMEA-000370-PIP02-18

OxThera AB; ICD10-E72.53 (hyperoxaluria) / Treatment of primary hyperoxaluria

Day 90 discussion

Action: For discussion

Endocrinology-Gynaecology-Fertility-Metabolism / Uro-nephrology

3.1.5. Humanised anti-CD19, fragment crystallisable (Fc) engineered, monoclonal antibody - Orphan - EMEA-002414-PIP01-18

Xencor, Inc.; Immunoglobulin G4-related disease / Treatment of adults, adolescents and children (> 23 months of age) with immunoglobulin G4-related disease

Day 90 discussion

Action: For discussion

Immunology-Rheumatology-Transplantation

3.1.6. Vedolizumab - EMEA-000645-PIP03-18

ICD-9-CM 279.51 / ICD-10-CM D89.810 - Other disorders involving the immune mechanism, not elsewhere classified: acute graft-versus-host disease

Day 90 discussion

Action: For discussion

Immunology-Rheumatology-Transplantation / Gastroenterology-Hepatology

3.1.7. Bulevirtide - Orphan - EMEA-002399-PIP01-18

MYR GmbH; Chronic hepatitis D infection

Day 90 discussion

Action: For discussion

Infectious Diseases

3.1.8. Recombinant adeno-associated viral vector serotype 2 carrying the gene for the human aromatic L-amino acid decarboxylase protein – Orphan - EMEA-002435-PIP01-18

PTC Therapeutic International Limited; Aromatic L-amino acid decarboxylase (AADC) deficiency / Treatment of aromatic L-AADC deficiency

Day 90 discussion

Action: For discussion

Neurology

3.1.9. EMEA-002446-PIP01-18

Ichthyosis associated with Sjögren-Larsson syndrome (SLS) / Treatment of ichthyosis associated with Sjögren-Larsson syndrome (SLS)

Day 90 discussion

Action: For discussion

Other / Endocrinology-Gynaecology-Fertility-Metabolism / Dermatology

3.1.10. EMEA-002327-PIP02-19

Treatment and prevention of oral mucositis

Day 60 discussion

Action: For discussion

Dermatology

3.1.11. Tezepelumab - EMEA-002579-PIP01-18

Atopic dermatitis

Day 60 discussion

Action: For discussion

Dermatology

3.1.12. EMEA-002552-PIP01-19

Treatment of non-alcoholic steatohepatitis (NASH)

Day 60 discussion

Action: For discussion

Gastroenterology-Hepatology

3.1.13. Hematopoietic stem cells modified with a lentiviral vector encoding for the human beta 2 integrin/CD18 gene - Orphan - EMEA-002562-PIP01-19

Rocket Pharmaceuticals, Inc.; Severe leukocyte adhesion deficiency type I (LAD-I)

Day 60 discussion

Action: For discussion

Immunology-Rheumatology-Transplantation

3.1.14. Obinutuzumab - Orphan - EMEA-001207-PIP02-19

Roche Registration GmbH; Systemic lupus erythemathosus

Day 60 discussion

Action: For discussion

Immunology-Rheumatology-Transplantation

3.1.15. EMEA-002563-PIP01-19

Treatment of focal epilepsy

Day 60 discussion

Action: For discussion

Neurology

3.1.16. 6-(2-hydroxy-2-methylpropoxy)-4-(6-(6-((6-methoxypyridin-3-yl)methyl)-3,6-diazabicyclo[3.1.1]heptan-3-yl)pyridin-3-yl)pyrazolo[1,5-a]pyridine-3-carbonitrile - Orphan - EMEA-002544-PIP01-18

Loxo Oncology, Inc.; Treatment of all conditions included in the category of malignant neoplasms (except haematopoietic and lymphoid tissue neoplasms) / Treatment of paediatric patients from ≥6 months to <18 years of age with rearranged during transfection (RET)-altered, locally advanced or metastatic, solid tumours or primary central nervous system (CNS) tumours

Day 60 discussion

Action: For discussion

Oncology

3.1.17. Belantamab mafodotin - Orphan - EMEA-002468-PIP03-19

GlaxoSmithKline Trading Services; Treatment of mature B-cell neoplasms / Treatment for adult patients with B-cell maturation antigen (BCMA)-expressing mature B-cell neoplasms

Day 60 discussion

Action: For discussion

Oncology

3.1.18. Carfilzomib - Orphan - EMEA-001806-PIP04-19

Amgen Europe BV; Treatment of acute lymphoblastic leukaemia (ALL) / Treatment of paediatric patients aged 1 year or older and young adult patients up to 21 years of age with bone marrow relapse of T-cell ALL treated with at least 1 prior therapy or B-cell ALL treated with at least 2 prior therapies, with or without extramedullary disease

Day 60 discussion

Action: For discussion

Oncology / Haematology-Hemostaseology

3.1.19. Atropine sulphate - EMEA-002538-PIP01-18

Treatment of myopia

Day 60 discussion

Action: For discussion

Ophthalmology

3.1.20. Brimonidine tartrate - EMEA-002558-PIP01-19

Conjunctival hyperaemia due to minor eye irritation

Day 60 discussion

Action: For discussion

Ophthalmology

3.1.21. Lonafarnib - Orphan - EMEA-002516-PIP01-18

Eiger BioPharmaceuticals Europe Limited; progeroid laminopathies, Hutchinson-Gilford progeria syndrome (HGPS)

Day 60 discussion

Action: For discussion

Other

3.1.22. Dexamethasone sodium phosphate / levofloxacin - EMEA-002375-PIP02-18

Acute otitis externa / Treatment of acute otitis externa

Day 60 discussion

Action: For discussion

Oto-rhino-laryngology

3.1.23. Dexamethasone sodium phosphate / levofloxacin - EMEA-002375-PIP03-18

Acute otitis media with spontaneous tympanic membrane perforation / Treatment of acute otitis media with spontaneous tympanic membrane perforation

Day 60 discussion

Action: For discussion

Oto-rhino-laryngology

3.1.24. Selonsertib - EMEA-001868-PIP04-18

Chronic kidney disease (CKD) / Treatment of patients with progressive CKD resulting from congenital anomalies of the kidney and urinary track (CAKUT) aged 3 to less than 18 years

Day 60 discussion

Action: For discussion

Uro-nephrology

3.1.25. Bordetella pertussis antigen: pertactin / bordetella pertussis antigen: filamentous haemagglutinin / bordetella pertussis antigen: pertussis toxoid / tetanus toxoid / diphtheria toxoid - EMEA-002343-PIP01-18

ICD10: A36 (diphtheria), ICD10: A37 (whooping cough), ICD10: A35 (other tetanus) / Active booster immunisation

Day 60 discussion

Action: For discussion

Vaccines / Infectious Diseases

3.1.26. Remimazolam - EMEA-001880-PIP02-19

Sedation during medical procedures / General anaesthesia and post-operative sedation up to 24hr, sedation in the intensive care unit (ICU), Sedation for short procedures

Day 30 discussion

Action: For discussion

Anaesthesiology

3.1.27. EMEA-002568-PIP01-19

Psoriasis / Treatment of moderate to severe chronic plaque-type psoriasis who are candidates for systemic therapy

Day 30 discussion

Action: For discussion

Dermatology

3.1.28. Botulinum toxin type A - EMEA-002521-PIP01-18

Muscle-induced wrinkles

Day 30 discussion

Action: For discussion

Dermatology

3.1.29. Hydrogen peroxide - EMEA-001884-PIP03-18

Treatment of common warts (verrucae vulgaris) / Topical treatment to remove common warts (verrucae vulgaris)

Day 30 discussion

Action: For discussion

Dermatology

3.1.30. Recombinant humanised anti-blood dendritic cell antigen 2 (BDCA2) monoclonal antibody - EMEA-002555-PIP01-19

Cutaneous lupus erythematosus

Day 30 discussion

Action: For discussion

Dermatology

3.1.31. EMEA-002577-PIP01-19

Prostate-specific membrane antigen (PSMA)-expressing metastatic prostate cancer

Day 30 discussion

Action: For discussion

Diagnostic / Oncology

3.1.32. Human chorionic gonadotrophin - EMEA-002547-PIP01-19

Infertility / Assisted reproductive technology (ART) programme such as in vitro fertilisation, anovulatory or oligo-ovulatory women

Day 30 discussion

Action: For discussion

Endocrinology-Gynaecology-Fertility-Metabolism

3.1.33. Mifepristone - EMEA-001437-PIP02-19

Endometriosis

Day 30 discussion

Action: For discussion

Endocrinology-Gynaecology-Fertility-Metabolism

3.1.34. Norethisterone acetate / estradiol / relugolix - EMEA-002428-PIP02-18

Endometriosis / Treatment of symptoms associated with endometriosis

Day 30 discussion

Action: For discussion

Endocrinology-Gynaecology-Fertility-Metabolism

3.1.35. Cilofexor - EMEA-002554-PIP01-19

Treatment of primary sclerosing cholangitis (PSC) (DB96.2)

Day 30 discussion

Action: For discussion

Gastroenterology-Hepatology

3.1.36. Dupilumab - EMEA-001501-PIP04-19

Treatment of eosinophilic esophagitis

Day 30 discussion

Action: For discussion

Gastroenterology-Hepatology

3.1.37. CD34^{+}enriched cells from patients with Fanconi anemia subtype A (FA-A) transduced ex vivo with lentiviral vector carrying the FANCA gene, PGKFANCA-WPRE - Orphan - EMEA-002578-PIP01-19

Rocket Pharmaceuticals, Inc.; Treatment of Fanconi anaemia subtype A

Day 30 discussion

Action: For discussion

Haematology-Hemostaseology

3.1.38. Probenecid / sulopenem etzadroxil - EMEA-002602-PIP01-19

Urinary tract infections / Abdominal and gastrointestinal infections / Uncomplicated urinary tract infections / Complicated intra-abdominal infections /Complicated urinary tract infections

Day 30 discussion

Action: For discussion

Infectious Diseases

3.1.39. Sulopenem - EMEA-002478-PIP01-18

Urinary Tract Infections/ Abdominal and gastrointestinal infections / Uncomplicated urinary tract infections / Complicated intra-abdominal infections / Complicated urinary tract infections

Day 30 discussion

Action: For discussion

Infectious Diseases

3.1.40. Istradefylline - EMEA-002540-PIP01-18

Parkinson's disease

Day 30 discussion

Action: For discussion

Neurology

3.1.41. EMEA-002575-PIP01-19

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

Day 30 discussion

Action: For discussion

Oncology

3.1.42. EMEA-002573-PIP01-19

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

Day 30 discussion

Action: For discussion

Oncology

3.1.43. Humanised fragment crystallisable (Fc) engineered monoclonal antibody against CD19 for the treatment of diffuse large B-cell lymphoma - Orphan - EMEA-002499-PIP02-19

MorphoSys AG; Diffuse large B-Cell lymphoma

Day 30 discussion

Action: For discussion

Oncology

3.1.44. Pracinostat - Orphan - EMEA-002567-PIP01-19

Helsinn Birex Pharmaceuticals limited; Acute myeloid leukaemia / ICD10 code C92.0

Day 30 discussion

Action: For discussion

Oncology

3.1.45. Ciclosporin - EMEA-002491-PIP02-19

Treatment of dry eye disease

Day 30 discussion

Action: For discussion

Ophthalmology

3.1.46. EMEA-002559-PIP01-19

Haemolytic disease of the foetus and newborn (HDFN)

Day 30 discussion

Action: For discussion

Other

3.1.47. Budesonide - Orphan - EMEA-002500-PIP01-18

Calliditas Therapeutics AB; Primary IgA nephropathy

Day 30 discussion

Action: For discussion

Uro-nephrology

3.2. Discussions on Compliance Check

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

3.2.1. Crizanlizumab - EMEA-C1-002141-PIP01-17-M01

Novartis Europharm Limited; Treatment of sickle cell disease

Day 30 discussion

Action: For discussion

Haematology-Hemostaseology

3.2.2. Efmoroctocog alfa - EMEA-C-001114-PIP01-10-M03

Swedish Orphan Biovitrum AB (publ); Treatment of hereditary factor VIII deficiency

Day 30 discussion

Action: For discussion

Haematology-Hemostaseology

3.2.3. Ustekinumab - EMEA-C-000311-PIP01-08-M04

Janssen-Cilag International NV; Treatment of plaque psoriasis

Day 30 discussion

Action: For discussion

Immunology-Rheumatology-Transplantation / Dermatology

3.2.4. Peginterferon alfa-2a - EMEA-C-000298-PIP01-08-M06

Roche Registration GmbH; Treatment of chronic hepatitis B

Day 30 discussion

Action: For discussion

Infectious Diseases

3.2.5. Rituximab - EMEA-C-000308-PIP01-08-M04

Roche Registration GmbH; Treatment of diffuse large B-cell lymphoma

Day 30 discussion

Action: For discussion

Oncology

3.3. Discussions on Modification of an Agreed Paediatric Investigation Plan

3.3.1. Apixaban - EMEA-000183-PIP01-08-M07

Bristol-Myers Squibb / Pfizer EEIG; Prevention of arterial thromboembolism (TE) / Prevention of venous thromboembolism / Prevention of TE in paediatric patients with cardiac disease / Prevention of venous thromboembolism (VTE) in paediatric subjects 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

Day 30 discussion

Action: For discussion

Cardiovascular Diseases

3.3.2. Fluciclovine (^{18}F) - Orphan - EMEA-001644-PIP02-14-M01

Blue Earth Diagnostics Ireland Ltd; Diagnosis of amino acid metabolism in solid malignant tumours / Diagnosis of primary and recurrent brain tumours

Day 30 discussion

Action: For discussion

Diagnostic / Oncology

3.3.3. Saxagliptin - EMEA-000200-PIP01-08-M08

AstraZeneca AB; E11 type 2 diabetes / Treatment of type 2 diabetes

Day 30 discussion

Action: For discussion

Endocrinology-Gynaecology-Fertility-Metabolism

3.3.4. Belatacept - EMEA-000157-PIP01-07-M04

Bristol-Myers Squibb Pharma EEIG; Prevention of rejection of transplanted kidney / in combination with corticosteroids and a mycophenolic acid (MPA), indicated for prophylaxis of graft rejection in pediatric patients at least 12 years of age and with a stable renal transplant for at least 6 months, who convert to a calcineurin inhibitor-free (CNI-free) maintenance immunosuppressive regimen

Day 30 discussion

Action: For discussion

Immunology-Rheumatology-Transplantation

3.3.5. Ixekizumab - EMEA-001050-PIP02-18-M01

Eli Lilly Nederland B.V.; 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 juvenile onset axial spondyloarthritis (JoAS)) and juvenile psoriatic arthritis

Day 30 discussion

Action: For discussion

Immunology-Rheumatology-Transplantation

3.3.6. Recombinant human monoclonal antibody to granulocyte-macrophage colony-stimulating factor (GM-CSF) - EMEA-001882-PIP02-16-M01

GlaxoSmithKline Trading Services Limited; 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 30 discussion

Action: For discussion

Immunology-Rheumatology-Transplantation

3.3.7. Delamanid - Orphan - EMEA-001113-PIP01-10-M06

Otsuka Pharmaceutical Development & Commercialisation Europe GmbH; Treatment of multi drug resistant tuberculosis

Day 30 discussion

Action: For discussion

Infectious Diseases

3.3.8. Eslicarbazepine acetate - EMEA-000696-PIP02-10-M06

BIAL - Portela & Ca, SA; Treatment of epilepsy with partial onset seizures / Treatment of epilepsy with partial onset seizures with eslicarbazepine acetate (ESL) as adjunctive therapy / Treatment of epilepsy with partial onset seizures with eslicarbazepine acetate (ESL) as monotherapy

Day 30 discussion

Action: For discussion

Neurology

3.3.9. Eteplirsen - Orphan - EMEA-001722-PIP01-14-M02

Sarepta Therapeutics Ireland Limited; Duchenne muscular dystrophy

Day 30 discussion

Action: For discussion

Neurology

3.3.10. Lasmiditan - EMEA-002166-PIP01-17-M02

Eli Lilly and Company Limited; Migraine with and without aura

Day 30 discussion

Action: For discussion

Neurology

3.3.11. Recombinant human tripeptidyl peptidase 1 (rhTPP1) - Orphan - EMEA-001362-PIP01-12-M04

BioMarin International Limited; neuronal ceroid lipofuscinosis type 2 (CLN2) disease / Treatment of CLN2

Day 30 discussion

Action: For discussion

Neurology

3.3.12. Bosutinib - EMEA-000727-PIP01-09-M03

Pfizer Europe MA EEIG; Treatment of chronic myeloid leukemia (CML) / Treatment of chronic, accelerated or blast phase CML with resistance or intolerance to prior tyrosine kinase inhibitor (TKI) therapy / Treatment of newly-diagnosed chronic phase of Philadelphia chromosome–positive chronic myeloid leukemia(Ph+ CML)

Day 30 discussion

Action: For discussion

Oncology

3.3.13. Daunorubicin (liposomal formulation) / cytarabine (liposomal formulation) - Orphan - EMEA-001858-PIP02-16-M03

Jazz Pharmaceuticals Ireland Limited; Acute myeloid leukaemia

Day 30 discussion

Action: For discussion

Oncology

3.3.14. Ixazomib - Orphan - EMEA-001410-PIP02-17-M02

Takeda Pharm A/S; Treatment of lymphoid malignancies (excluding multiple myeloma) / Treatment of multiple myeloma (MM) / Treatment of adult patients with newly diagnosed multiple myeloma (NDMM) / Treatment of paediatric patients diagnosed with relapsed precursor B lineage-acute lymphoblastic leukaemia (B-ALL) or T-ALL

Day 30 discussion

Action: For discussion

Oncology

3.3.15. Selumetinib - Orphan - EMEA-001585-PIP01-13-M03

AstraZeneca AB; Treatment of thyroid cancer / Treatment of neurofibromatosis type 1 / selumetinib in combination with adjuvant radioactive iodine therapy is indicated 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

neurofibromatosis type 1 (NF1) related plexiform neurofibroma in children and adolescents

Day 30 discussion

Action: For discussion

Oncology

3.3.16. Palonosetron / fosnetupitant - EMEA-001198-PIP03-17-M02

Helsinn Birex Pharmaceuticals Limited; Prevention of chemotherapy-induced nausea and vomiting

Day 30 discussion

Action: For discussion

Other

3.3.17. Octenidine - EMEA-001514-PIP01-13-M01

Cassella-med GmbH & Co. KG; Treatment of upper respiratory tract infections / Treatment of sore-throat due to infectious pharyngitis

Day 30 discussion

Action: For discussion

Oto-rhino-laryngology

3.3.18. Reslizumab - EMEA-001202-PIP02-13-M03

Teva Pharmaceuticals Europe; Treatment of asthma / Add-on treatment to reduce exacerbations, relieve symptoms and improve lung function in paediatric patients from 6 to less than 18 years of age with inadequately controlled severe asthma who have a blood eosinophil count greater than equal to 300 microlitre

Day 30 discussion

Action: For discussion

Pneumology - Allergology

3.3.19. Tezepelumab - EMEA-001613-PIP01-14-M03

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

3.3.20. Mirabegron - EMEA-000597-PIP02-10-M07

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

Day 30 discussion

Action: For discussion

Uro-nephrology

3.3.21. Recombinant influenza hemagglutinin-strain B (Yamagata lineage) / Recombinant influenza hemagglutinin-strain B (Victoria lineage) / Recombinant influenza hemagglutinin-strain A (H3N2 subtype) / Recombinant influenza hemagglutinin-strain A (H1N1 subtype) - EMEA-002418-PIP01-18-M01

Sanofi Pasteur; Prevention of influenza infection

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 2019 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.

5.1. New Scientific Advice

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

5.2. Ongoing Scientific Advice

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

5.3. Final Scientific Advice (Reports and Scientific Advice letters)

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. Anti-vascular endothelial growth factor/ angiopoietin-2 (VEGF/Ang2) nanobody) - EMEA-06-2019

Boehringer Ingelheim International GmbH; All classes of medicinal products for treatment of age-related macular degeneration and diabetic macular oedema / indicated for treatment of patients with wet age-related macular degeneration and diabetic macular edema

Action: For adoption

6.1.2. Enzalutamide - EMEA-07-2019

Astellas Pharma Europe B.V.; The classes of androgen receptor modulator / oestrogen receptor modulator / growth and sex hormone as well as their releasing or inhibiting factors, and of sex hormone-metabolism modulator medicinal products for treatment of breast malignant neoplasms, prostate malignant neoplasms and neuroendocrine malignant

neoplasms / Treatment of adult men with metastatic hormone-sensitive prostate cancer (mHSPC) in combination with androgen deprivation therapy

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. Fragment crystallisable (Fc)- and complementarity-determining regions (CDR)-modified humanised monoclonal antibody against C5 - EMEA-001943-PIP01-16-M01

Alexion Pharma GmbH; Treatment of atypical haemolytic uremic Syndrome

Proposed indication: Treatment of adult patients with complement-mediated thrombotic microangiopathy (TMA)

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 Annex B.

9. Organisational, regulatory and methodological matters

9.1. Mandate and organisation of the PDCO

PDCO member(s)/alternate(s) mandate status

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. Concept paper on the need for revision of the guideline on the investigation of medicinal products in the term and preterm neonate

Action: For adoption

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

No items

9.5. Cooperation with International Regulators

No items

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

9.6.1. 4th Accelerate Paediatric Strategy forum on acute myeloid leukaemia (AML)

Action: For information

9.7. PDCO work plan

No items

9.8. Planning and reporting

No items

10. Any other business

No items

11. Breakout sessions

11.1.1. Paediatric oncology

Action: For discussion on Thursday, 14:00 - 15:00, room 1-B

11.1.2. Neonatology

Action: For discussion on Thursday, 14:00 - 15:00, room 0-E

11.1.3. Inventory

Action: For discussion on Thursday, 14:00 - 15:00, room 0-F

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/