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SCIENCE MEDICINES HEALTH

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Inspections, Human Medicines Pharmacovigilance and Committees Division

Paediatric Committee (PDCO)

Draft agenda for the meeting on 28-31 January 2020

Chair: Koenraad Norga – Vice-Chair: Sabine Scherer

28 January 2020, 14:00- 19:00, room 2C

29 January 2020, 08:30- 19:00, room 2C

30 January 2020, 08:30- 19:00, room 2C

31 January 2020, 08:30- 13:00, room 2C

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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 ongoing 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 28-31 January 2020. See 28-31 January 2020 PDCO minutes (to be published post 25-28 February 2020 PDCO meeting).

1.2. Adoption of agenda

PDCO agenda for 28-31 January 2020

1.3. Adoption of the minutes

PDCO minutes for 09-11 December 2019

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. EMEA-002481-PIP01-18

Atopic dermatitis

Day 120 opinion

Action: For adoption

Dermatology

2.1.2. EMEA-002552-PIP01-19

Treatment of non-alcoholic steatohepatitis (NASH)

Day 120 opinion

Action: For adoption

Gastroenterology-Hepatology

2.1.3. Avatrombopag maleate - EMEA-001136-PIP02-19

Chemotherapy-induced thrombocytopenia / Treatment of chemotherapy-induced thrombocytopenia (CIT) in patients receiving myelosuppressive chemotherapy for solid tumours.

Day 120 opinion

Action: For adoption

Haematology-Hemostaseology

2.1.4. CD34+ enriched cells from patients with Fanconi anaemia subtype A (FA-A) transduced ex vivo with lentiviral vector carrying the FANCA gene - Orphan - EMEA-002578-PIP01-19

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

Day 120 opinion

Action: For adoption

Haematology-Hemostaseology

2.1.5. Allogeneic, non-expanded, umbilical cord blood-derived, haematopoietic mature myeloid and lymphoid cells (NF) / Allogeneic, ex vivo expanded, umbilical cord blood-derived, haematopoietic CD34+ progenitor cells (CF) - Orphan - EMEA-001913-PIP02-18

Gamida Cell Ltd; Treatment in haematopoietic stem cell transplantation / Haematopoietic reconstitution of patients who are medically indicated for allogeneic haematopoietic stem cell transplantation

Day 120 opinion

Action: For adoption

Immunology-Rheumatology-Transplantation

2.1.6. Baricitinib - EMEA-001220-PIP05-19

Treatment of systemic lupus erythematosus (SLE)

Day 120 opinion

Action: For adoption

Immunology-Rheumatology-Transplantation

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

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

Day 120 opinion

Action: For adoption

Immunology-Rheumatology-Transplantation

2.1.8. Zoliflodacin - EMEA-002599-PIP01-19

Treatment of gonococcal infection / Treatment of uncomplicated gonorrhoea

Day 120 opinion

Action: For adoption

Infectious Diseases

2.1.9. Cannabidiol - Orphan - EMEA-001964-PIP02-19

GW Pharma (International) B.V; Treatment of Rett syndrome

Day 120 opinion

Action: For adoption

Neurology

2.1.10. Efgartigimod alfa - Orphan - EMEA-002597-PIP01-19

argenx BVBA; Treatment of myasthenia gravis

Day 120 opinion

Action: For adoption

Neurology

2.1.11. EMEA-002572-PIP01-19

Chromosome 15q duplication syndrome / Cyclin-dependent kinase-like 5 (CDKL5) deficiency disorder / Treatment of seizures associated with chromosome 15q duplication syndrome / Treatment of seizures associated with CDKL5 deficiency disorder

Day 120 opinion

Action: For adoption

Neurology

2.1.12. Timrepigene emparvovec - Orphan - EMEA-002430-PIP01-18

Nightstar Europa Limited; Treatment of choroideremia

Day 120 opinion

Action: For adoption

Ophthalmology

2.1.13. [Anti-neonatal Fc receptor human monoclonal antibody - Orphan - EMEA-002559-PIP02-19](#)

Momenta Pharmaceuticals, Inc.; Myasthenia gravis

Day 120 opinion

Action: For adoption

Other

2.1.14. [Ezetimibe / Rosuvastatin - EMEA-002257-PIP02-19](#)

Prevention of cardiovascular events / Rosuvastatin/Ezetimibe is indicated to reduce the risk of cardiovascular events as substitution therapy in patients with coronary heart disease (CHD) and a history of acute coronary syndrome (ACS), who are adequately controlled with the individual substances given concurrently at the same dose level as in the fixed dose combination, but as separate products

Day 60 opinion

Action: For adoption

Cardiovascular Diseases

2.1.15. [Nemolizumab - EMEA-001624-PIP02-19](#)

Prurigo nodularis

Day 60 opinion

Action: For adoption

Dermatology

2.1.16. [Gefapixant - EMEA-002267-PIP03-19](#)

Treatment of endometriosis

Day 60 opinion

Action: For adoption

Endocrinology-Gynaecology-Fertility-Metabolism

2.1.17. [Carbidopa / levodopa - EMEA-002687-PIP01-19](#)

Parkinson's disease

Day 60 opinion

Action: For adoption

Neurology

2.1.18. Elezanumab - EMEA-002675-PIP01-19

Multiple sclerosis

Day 60 opinion

Action: For adoption

Neurology

2.1.19. Verdiperstat - Orphan - EMEA-002708-PIP01-19

Biohaven Pharmaceuticals, Inc.; Multiple system atrophy

Day 60 opinion

Action: For adoption

Neurology

2.1.20. EMEA-002686-PIP01-19

Treatment of breast cancer

Day 60 opinion

Action: For adoption

Oncology

2.1.21. EMEA-002690-PIP01-19

Treatment of non-small cell lung cancer

Day 60 opinion

Action: For adoption

Oncology

2.1.22. Daratumumab - Orphan - EMEA-002152-PIP03-19

Janssen-Cilag International N.V.; Systemic light chain amyloidosis

Day 60 opinion

Action: For adoption

Oncology

2.1.23. Eftilagimod alpha - EMEA-002698-PIP01-19

Treatment of breast tumours / Treatment of benign breast tumours / Treatment of primary breast cancer / Treatment of secondary breast cancer (primary cancer metastasizing to the breast)

Day 60 opinion

Action: For adoption

Oncology

2.1.24. Parsaclisib - Orphan - EMEA-002696-PIP01-19

Incyte Biosciences Distribution B.V.; Treatment of mature B-cell neoplasm

Day 60 opinion

Action: For adoption

Oncology

2.1.25. Zolbetuximab - Orphan - EMEA-002695-PIP01-19

Astellas Pharma Europe B.V.; Treatment of gastric and gastroesophageal junction adenocarcinoma / Treatment of pancreatic adenocarcinoma

Day 60 opinion

Action: For adoption

Oncology

2.1.26. Brolucizumab - EMEA-002691-PIP01-19

Central retinal vein occlusion (CRVO) / Branch retinal vein occlusion (BRVO)

Day 60 opinion

Action: For adoption

Ophthalmology

2.1.27. EMEA-002668-PIP01-19

Treatment of chronic pain in patients, who have not sufficiently responded to different analgesic therapies including at least one opioid (adult population only)

Day 60 opinion

Action: For adoption

Pain

2.2. Opinions on Compliance Check

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

2.2.1. Filgotinib - EMEA-C1-001619-PIP03-16

Gilead Sciences International Ltd.; Treatment of ulcerative colitis / Treatment of Crohn's disease

Day 60 letter

Action: For adoption

Gastroenterology-Hepatology

2.2.2. Autologous cartilage derived cultured chondrocytes - EMEA-C1-001823-PIP01-15-M01

TETEC AG; Treatment of cartilage disorders

Day 60 letter

Action: For adoption

Other

2.3. Opinions on Modification of an Agreed Paediatric Investigation Plan

2.3.1. Entrectinib - EMEA-002096-PIP01-16-M02

Roche Registration GmbH; Treatment of all conditions included in the category of malignant neoplasms (except haematopoietic and lymphoid tissue neoplasms) / For the treatment of paediatric patients with neurotrophic tyrosine receptor kinase (NTRK) fusion-positive solid tumours

Day 60 opinion

Action: For adoption

2.3.2. Ixekizumab - EMEA-001050-PIP01-10-M05

Eli Lilly Nederland B.V.; Treatment of psoriasis

Day 60 opinion

Action: For adoption

Dermatology

2.3.3. [Nemolizumab - EMEA-001624-PIP01-14-M02](#)

Galderma International S.A.; Atopic dermatitis / Treatment of moderate to severe atopic dermatitis not adequately controlled with topical treatments

Day 60 opinion

Action: For adoption

Dermatology

2.3.4. [Alogliptin benzoate \(as alogliptin\) - EMEA-000496-PIP01-08-M07](#)

Takeda Development Centre Europe Ltd; Type 2 diabetes mellitus

Day 60 opinion

Action: For adoption

Endocrinology-Gynaecology-Fertility-Metabolism

2.3.5. [Dapagliflozin - EMEA-000694-PIP01-09-M08](#)

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

Day 60 opinion

Action: For adoption

Endocrinology-Gynaecology-Fertility-Metabolism

2.3.6. [Denosumab - EMEA-000145-PIP02-12-M02](#)

Amgen Europe B.V.; Treatment of osteoporosis / Treatment of osteogenesis imperfect / Treatment of glucocorticoid induced osteoporosis

Day 60 opinion

Action: For adoption

Endocrinology-Gynaecology-Fertility-Metabolism

2.3.7. [Evinacumab - EMEA-002298-PIP01-17-M01](#)

Regeneron Ireland DAC; Treatment of elevated cholesterol / Treatment of homozygous familial hypercholesterolemia (HoFH)

Day 60 opinion

Action: For adoption

Endocrinology-Gynaecology-Fertility-Metabolism

2.3.8. Sodium zirconium cyclosilicate - EMEA-001539-PIP01-13-M04

AstraZeneca AB; Hyperkalemia / Treatment of hyperkalemia

Day 60 opinion

Action: For adoption

Endocrinology-Gynaecology-Fertility-Metabolism

2.3.9. Macimorelin - EMEA-001988-PIP01-16-M01

Aeterna Zentaris GmbH; Growth hormone deficiency / Diagnosis of growth hormone deficiency

Day 60 opinion

Action: For adoption

Endocrinology-Gynaecology-Fertility-Metabolism / Diagnostic

2.3.10. Eluxadoline - EMEA-001579-PIP01-13-M03

Allergan Pharmaceuticals International Limited; Irritable bowel syndrome with diarrhoea

Day 60 opinion

Action: For adoption

Gastroenterology-Hepatology

2.3.11. Eftrenonacog alfa - Orphan - EMEA-000914-PIP01-10-M05

Swedish Orphan Biovitrum AB (publ); Hereditary factor IX deficiency - D67

Day 60 opinion

Action: For adoption

Haematology-Hemostaseology

2.3.12. Vadadustat - EMEA-001944-PIP01-16-M01

Akebia Therapeutics, Inc.; Treatment of anaemia due to chronic disorders / Treatment of anaemia secondary to chronic kidney disease

Day 60 opinion

Action: For adoption

Haematology-Hemostaseology

2.3.13. Avacopan - Orphan - EMEA-002023-PIP01-16-M04

ChemoCentryx Ireland Ltd.; Treatment of antineutrophil cytoplasmic antibody (ANCA)-associated vasculitis

Day 60 opinion

Action: For adoption

Immunology-Rheumatology-Transplantation

2.3.14. Emapalumab - Orphan - EMEA-002031-PIP01-16-M03

Novimmune BV; Treatment of haemophagocytic lymphohistiocytosis

Day 60 opinion

Action: For adoption

Immunology-Rheumatology-Transplantation

2.3.15. Bezlotoxumab - EMEA-001645-PIP01-14-M03

Merck Sharp & Dohme (Europe), Inc.; Treatment of clostridium difficile infection / Indicated for the prevention of recurrence of clostridium difficile infection (CDI) in paediatric patients at high risk for recurrence of CDI

Day 60 opinion

Action: For adoption

Infectious Diseases

2.3.16. Nirsevimab -anti-respiratory syncytial virus human IgG1k monoclonal antibody (MEDI8897) - EMEA-001784-PIP01-15-M02

AstraZeneca AB; Prevention of respiratory syncytial viral infections

Day 60 opinion

Action: For adoption

Infectious Diseases

2.3.17. Posaconazole - EMEA-000468-PIP02-12-M06

Merck Sharp & Dohme (Europe), Inc.; Prevention of invasive fungal infections / Treatment of invasive fungal infections / For 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

2.3.18. Tenofovir alafenamide - EMEA-001584-PIP01-13-M05

Gilead Sciences International Ltd.; Treatment of chronic Hepatitis B / Vemlidy is 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

2.3.19. Eculizumab - Orphan - EMEA-000876-PIP03-14-M04

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

Day 60 opinion

Action: For adoption

Neurology

2.3.20. Eladocagene exuparvovec - Orphan - EMEA-002435-PIP01-18-M01

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

Day 60 opinion

Action: For adoption

Neurology

2.3.21. Ozanimod - EMEA-001710-PIP02-14-M04

Celgene Europe B.V.; Treatment of multiple sclerosis / Treatment of relapsing remitting forms of multiple sclerosis

Day 60 opinion

Action: For adoption

Neurology

2.3.22. Perampanel - EMEA-000467-PIP01-08-M13

Eisai Europe Limited; Treatment of treatment-resistant epilepsies / Adjunctive therapy in patients with other paediatric epilepsies / Adjunctive therapy in patients with refractory partial onset seizures including secondarily generalised seizures

Day 60 opinion

Action: For adoption

Neurology

2.3.23. Risdiplam - Orphan - EMEA-002070-PIP01-16-M04

Roche Registration GmbH; Treatment of spinal muscular atrophy

Day 60 opinion

Action: For adoption

Neurology

2.3.24. Idasanutlin - Orphan - EMEA-001489-PIP01-13-M02

Roche Registration GmbH; Treatment of all conditions included in the category of malignant neoplasms (except nervous system, haematopoietic and lymphoid tissue) / Treatment of acute lymphoblastic leukaemia / Treatment of acute myeloid leukaemia / Treatment of children with a solid malignant tumour and which is newly-diagnosed and metastatic, or refractory to first-line treatment / Treatment of children with first relapse of, or with frontline-refractory acute myeloid leukaemia / Treatment of children with first relapse of, or with frontline-refractory acute lymphoblastic leukaemia

Day 60 opinion

Action: For adoption

Oncology

2.3.25. Sonidegib - EMEA-000880-PIP02-11-M04

Sun Pharmaceutical Industries Europe B.V.; Basal cell carcinoma / Medulloblastoma

Day 60 opinion

Action: For adoption

Oncology

2.3.26. Burosumab - Human recombinant IgG1 monoclonal antibody to fibroblast growth factor 23 (FGF23) - Orphan - EMEA-001659-PIP01-15-M04

Kyowa Kirin Holdings B.V.; X-linked hypophosphataemia / Treatment of X-linked hypophosphataemia

Day 60 opinion

Action: For adoption

Other

2.3.27. [Meloxicam / bupivacaine - EMEA-002246-PIP01-17-M01](#)

Heron Therapeutics B.V.; Acute postoperative pain

Day 60 opinion

Action: For adoption

Pain

2.3.28. [Ravulizumab \(ALXN1210\) - Orphan - EMEA-001943-PIP01-16-M04](#)

Alexion Europe SAS; Atypical haemolytic uremic syndrome / Treatment of atypical haemolytic uremic syndrome

Day 60 opinion

Action: For adoption

Uro-nephrology / Haematology-Hemostaseology

2.3.29. [Influenza virus surface antigens \(haemagglutinin and neuraminidase\) of strain B \(Victoria lineage\) / Influenza virus surface antigens \(haemagglutinin and neuraminidase\) of strain B \(Yamagata lineage\) / Influenza virus surface antigens \(haemagglutinin and neuraminidase\) of strain A \(H3N2\) / Influenza virus surface antigens \(haemagglutinin and neuraminidase\) of strain A \(H1N1\) - EMEA-002068-PIP01-16-M03](#)

Seqirus UK Limited; Influenza / Prevention of influenza

Day 60 opinion

Action: For adoption

Vaccines

2.3.30. [Live, attenuated, chimeric dengue virus, serotype 4 / Live, attenuated, chimeric dengue virus, serotype 3 / Live, attenuated, chimeric dengue virus, serotype 2 / Live, attenuated, chimeric dengue virus, serotype 1 - EMEA-001545-PIP01-13-M02](#)

Sanofi Pasteur; Prevention of dengue disease

Day 60 opinion

Action: For adoption

Vaccines

2.3.31. Split influenza virus, inactivated containing antigens equivalent to the B-like strain (Yamagata lineage) / Split influenza virus, inactivated containing antigens equivalent to the B-like strain (Victoria lineage) / Split influenza virus, inactivated containing antigens equivalent to the A/H3N2-like strain / Split influenza virus, inactivated containing antigens equivalent to the A/H1N1-like strain - EMEA-002359-PIP01-18-M01

Sanofi Pasteur; Prevention of influenza infection

Day 60 opinion

Action: For adoption

Vaccines

2.4. Opinions on Re-examinations

2.4.1. Inotuzumab ozogamicin - Orphan - EMEA-001429-PIP01-13-M03

Pfizer Europe MA EEIG; Treatment of acute lymphoblastic leukaemia / For the treatment of relapsed or refractory B cell precursor acute lymphoblastic leukaemia

Day 30 opinion

Action: For adoption

Oncology / Haematology-Hemostaseology

2.4.2. Vosoritide - Orphan - EMEA-002033-PIP01-16-M01

BioMarin International Limited; Treatment of achondroplasia

Day 30 opinion

Action: For adoption

Other

2.4.3. Dupilumab - EMEA-001501-PIP04-19

Treatment of eosinophilic esophagitis

Day 30 opinion

Action: For adoption

Gastroenterology-Hepatology

2.4.4. (R)-1-(3-(aminomethyl) phenyl)-N-(5-((3 cyanophenyl)(cyclopropylmethylamino)methyl)-2-fluorophenyl)-3-(trifluoromethyl)-1H-pyrazole-5-carboxamide dihydrochloride - Orphan - EMEA-002449-PIP02-18

BioCryst UK; Treatment of hereditary angioedema (HAE) / Treatment of HAE attacks/
Prevention of HAE attacks

Day 30 opinion

Action: For adoption

Pneumology - Allergology

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. Selpercatinib - EMEA-C1-002544-PIP01-18

Eli Lilly and Company; Treatment of all conditions included in the category of malignant neoplasms (except haematopoietic and lymphoid tissue neoplasms)

Day 1 Letter

Action: For information

Oncology

2.7.2. Mometasone (furoate) / Indacaterol (acetate) - EMEA-C3-001217-PIP01-11-M05

Novartis Europharm Ltd; Treatment of asthma

Day 1 letter

Action: For information

Pneumology – Allergology

2.7.3. Recombinant varicella zoster virus (VZV) glycoprotein E - EMEA-C1-001426-PIP01-13-M02

GlaxoSmithKline Biologicals SA; Prevention of varicella zoster virus (VZV) reactivation / Prevention of herpes zoster in immunocompromised subjects

Day 1 letter

Action: For information

Vaccines

2.7.4. Idecabtagene vicleucel - EMEA-C1-002369-PIP01-18-M01

Celgene Europe B.V.; Treatment of mature B-cell neoplasms

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. Lenadogene nolparvovec - Orphan - EMEA-001992-PIP02-16

GenSight-Biologics; Leber hereditary optic neuropathy (LHON)

Day 90 discussion

Action: For discussion

3.1.2. Dihomo- γ -linolenic acid (DGLA) - EMEA-002364-PIP03-19

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

Day 90 discussion

Action: For discussion

Dermatology

3.1.3. EMEA-002327-PIP02-19

Prevention of oral mucositis

Day 90 discussion

Action: For discussion

Dermatology

3.1.4. EMEA-002582-PIP01-19

Treatment of chronic spontaneous urticaria

Day 90 discussion

Action: For discussion

Dermatology

3.1.5. Tezepelumab - EMEA-002579-PIP01-18

Atopic dermatitis

Day 90 discussion

Action: For discussion

Dermatology

3.1.6. Ladarixin - EMEA-002642-PIP01-19

Treatment of type 1 diabetes / Treatment of new-onset type 1 diabetes mellitus

Day 90 discussion

Action: For discussion

Endocrinology-Gynaecology-Fertility-Metabolism

3.1.7. EMEA-001312-PIP02-19

Prevention of acute graft-versus-host disease (GVHD)

Day 90 discussion

Action: For discussion

Immunology-Rheumatology-Transplantation

3.1.8. Polymyxin B - EMEA-002595-PIP01-19

Treatment of infections due to aerobic Gram-negative bacteria

Day 90 discussion

Action: For discussion

Infectious Diseases

3.1.9. 5-[[4-[2-[5-(1-Hydroxyethyl)-2-pyridinyl] ethoxy] phenyl] methyl]-2,4-thiazolidinedione hydrochloride - Orphan - EMEA-002106-PIP01-16

Minoryx Therapeutics S.L.; Treatment of adrenoleukodystrophy (ALD) / Treatment of adrenomyeloneuropathy (AMN)

Day 90 discussion

Action: For discussion

Neurology

3.1.10. 1-[4-bromo-5-[1-ethyl-7-(methylamino)-2-oxo-1,2-dihydro-1,6-naphthyridin-3-yl]-2-fluorophenyl]-3-phenylurea - Orphan - EMEA-002526-PIP02-19

Deciphera Pharmaceuticals LLC; Treatment of gastrointestinal stromal tumours

Day 90 discussion

Action: For discussion

Oncology

3.1.11. Bintrafusp alfa - Orphan - EMEA-002586-PIP01-19

Merck Europe B.V.; Treatment of all conditions included in the category of malignant neoplasms (except central nervous system tumours, haematopoietic and lymphoid tissue neoplasms) / Treatment of paediatric patients from 3 to <18 years of age with solid malignant tumors

Day 90 discussion

Action: For discussion

Oncology

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

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

Day 90 discussion

Action: For discussion

Oncology

3.1.13. Immunoglobulin G4 - EMEA-002290-PIP01-17

Treatment of all conditions included in the category of malignant neoplasms (except central nervous system tumours, haematopoietic and lymphoid tissue neoplasms) / In combination with nivolumab for the treatment of malignant solid tumours in paediatric patients from 6 months to less than 18 years old.

Day 90 discussion

Action: For discussion

Oncology

3.1.14. 2-[[2-ethyl-6-[4-[2-(3-hydroxyazetid-1-yl)-2-oxoethyl]piperazin-1-yl]-8-methylimidazo[1,2-a]pyridin-3-yl](methylamino)-4-(4-fluorophenyl)-1,3-thiazole-5-carbonitrile - Orphan - EMEA-002333-PIP02-19

Galapagos NV; Treatment of idiopathic pulmonary fibrosis / Treatment of interstitial lung disease with fibrosis in children

Day 90 discussion

Action: For discussion

Pneumology - Allergology

3.1.15. [Tosatouxumab - Orphan - EMEA-002506-PIP01-18](#)

Aridis Pharmaceuticals Inc.; Pneumonia caused by staphylococcus aureus

Day 90 discussion

Action: For discussion

Pneumology - Allergology

3.1.16. [EMEA-002121-PIP03-19](#)

Treatment of insomnia / Treatment of insomnia in children with comorbid neurodevelopmental and psychiatric disorders

Day 90 discussion

Action: For discussion

Psychiatry

3.1.17. [EMEA-002641-PIP01-19](#)

Prevention of pneumococcal disease caused by *S. pneumoniae* / For the active immunisation for the prevention of invasive pneumococcal diseases (IPD) caused by *S. pneumoniae* in infants, children and adolescents from 6 weeks to < 18 years of age

Day 90 discussion

Action: For discussion

Vaccines

3.1.18. [Pneumococcal Polyssacharide Serotype 33F conjugated to CRM197 carrier protein and adsorbed on aluminium Phosphate / Pneumococcal Polyssacharide Serotype 23F conjugated to CRM197 carrier protein and adsorbed on aluminium Phosphate / Pneumococcal Polyssacharide Serotype 22F conjugated to CRM197 carrier protein and adsorbed on aluminium Phosphate / Pneumococcal Polyssacharide Serotype 19F conjugated to CRM197 carrier protein and adsorbed on aluminium Phosphate / Pneumococcal Polyssacharide Serotype 19A conjugated to CRM197 carrier protein and adsorbed on aluminium Phosphate / Pneumococcal Polyssacharide Serotype 18C conjugated to CRM197 carrier protein and adsorbed on aluminium Phosphate / Pneumococcal Polyssacharide Serotype 15B conjugated to CRM197 carrier protein and adsorbed on aluminium Phosphate / Pneumococcal Polyssacharide Serotype 14 conjugated to CRM197 carrier protein and adsorbed on aluminium Phosphate / Pneumococcal Polyssacharide Serotype 12F conjugated to CRM197 carrier protein and adsorbed on aluminium Phosphate / Pneumococcal Polyssacharide Serotype 11A conjugated to CRM197 carrier protein and adsorbed on aluminium Phosphate / Pneumococcal Polyssacharide Serotype 10A conjugated to CRM197 carrier protein and adsorbed on aluminium Phosphate / Pneumococcal Polyssacharide Serotype 9V conjugated to CRM197 carrier protein and adsorbed on aluminium Phosphate /](#)

Pneumococcal Polyssacharide Serotype 8 conjugated to CRM197 carrier protein and adsorbed on aluminium Phosphate / Pneumococcal Polyssacharide Serotype 7F conjugated to CRM197 carrier protein and adsorbed on aluminium Phosphate / Pneumococcal Polyssacharide Serotype 6B conjugated to CRM197 carrier protein and adsorbed on aluminium Phosphate / Pneumococcal Polyssacharide Serotype 6A conjugated to CRM197 carrier protein and adsorbed on aluminium Phosphate / Pneumococcal Polyssacharide Serotype 5 conjugated to CRM197 carrier protein and adsorbed on aluminium Phosphate / Pneumococcal Polyssacharide Serotype 4 conjugated to CRM197 carrier protein and adsorbed on aluminium Phosphate / Pneumococcal Polyssacharide Serotype 3 conjugated to CRM197 carrier protein and adsorbed on aluminium Phosphate / Pneumococcal Polyssacharide Serotype 1 conjugated to CRM197 carrier protein and adsorbed on aluminium Phosphate - EMEA-002330-PIP01-18

Disease caused by Streptococcus pneumoniae

Day 90 discussion

Action: For discussion

Vaccines

3.1.19. Temozolomide - EMEA-002634-PIP01-19

Treatment of malignant glioma / Children from the age of three years and adolescent patients with malignant glioma, such as glioblastoma multiform or anaplastic astrocytoma, showing recurrence or progression after standard therapy, who have difficulty swallowing

Day 90 discussion

Action: For discussion

Oncology

3.1.20. Pegcetacoplan - Orphan - EMEA-002600-PIP01-19

Apellis Ireland Limited; Paroxysmal nocturnal haemoglobinuria

Day 90 discussion

Action: For discussion

Haematology-Hemostaseology

3.1.21. Cenobamate - EMEA-002563-PIP02-19

Treatment of epilepsy

Day 90 discussion

Action: For discussion

Neurology

3.1.22. [Bilayer, engineered, collagen hydrogel-based skin graft composed of autologous keratinocytes and fibroblasts - Orphan - EMEA-002699-PIP01-19](#)

CUTISS AG; Treatment of burns

Day 60 discussion

Action: For discussion

Dermatology

3.1.23. [EMEA-002689-PIP01-19](#)

Achondroplasia

Day 60 discussion

Action: For discussion

Endocrinology-Gynaecology-Fertility-Metabolism

3.1.24. [Lonapegsomatropin - EMEA-002692-PIP01-19](#)

Growth hormone deficiency

Day 60 discussion

Action: For discussion

Endocrinology-Gynaecology-Fertility-Metabolism

3.1.25. [Cilofexor - Orphan - EMEA-002554-PIP02-19](#)

Gilead Sciences International Ltd.; Treatment of primary sclerosing cholangitis (PSC) (DB96.2)

Day 60 discussion

Action: For discussion

Gastroenterology-Hepatology

3.1.26. [Odevixibat - Orphan - EMEA-002054-PIP02-18](#)

Albireo AB; Biliary atresia

Day 60 discussion

Action: For discussion

Gastroenterology-Hepatology

3.1.27. [2-\(2-{{2-\(1H-benzimidazol-2-yl\) ethyl} amino} ethyl\)-N-\[\(3-fluoropyridin-2-yl\) methyl\]-1,3-oxazole-4-carboxamide trihydrochloride - Orphan - EMEA-002704-PIP01-19](#)

Vifor France; Beta-thalassaemia / Treatment of beta-thalassaemia

Day 60 discussion

Action: For discussion

Haematology-Hemostaseology

3.1.28. [Efgartigimod alfa - EMEA-002597-PIP02-19](#)

Treatment of immune thrombocytopenia

Day 60 discussion

Action: For discussion

Haematology-Hemostaseology

3.1.29. [Marstacimab - Orphan - EMEA-002285-PIP02-19](#)

Pfizer Europe MA EEIG; Treatment of haemophilia B / Treatment of haemophilia A / Prophylaxis of bleeding in haemophilia B / Prophylaxis of bleeding in haemophilia A

Day 60 discussion

Action: For discussion

Haematology-Hemostaseology

3.1.30. [Mitapivat - EMEA-002684-PIP01-19](#)

Pyruvate kinase deficiency / Treatment of paediatric patients with pyruvate kinase deficiency

Day 60 discussion

Action: For discussion

Haematology-Hemostaseology

3.1.31. [Allogeneic bone marrow derived mesenchymal stromal cells, ex-vivo expanded - Orphan - EMEA-002706-PIP01-19](#)

medac Gesellschaft für klinische Spezialpräparate mbH; Treatment of acute graft-versus-host disease

Day 60 discussion

Action: For discussion

Immunology-Rheumatology-Transplantation

3.1.32. Dapirolizumab pegol - EMEA-002702-PIP01-19

Treatment of systemic lupus erythematosus (SLE) / Treatment of children and adolescents ≥7 years to <18 years of age with active SLE despite standard therapy

Day 60 discussion

Action: For discussion

Immunology-Rheumatology-Transplantation

3.1.33. Rilzabrutinib - Orphan - EMEA-002438-PIP02-19

Principia Biopharma, Inc.; Immune thrombocytopenia / Treatment of immune thrombocytopenia

Day 60 discussion

Action: For discussion

Immunology-Rheumatology-Transplantation

3.1.34. Artesunate - EMEA-002710-PIP01-19

Malaria

Day 60 discussion

Action: For discussion

Infectious Diseases

3.1.35. EMEA-002694-PIP01-19

Treatment of chronic viral hepatitis B / Treatment of chronic hepatitis B infection

Day 60 discussion

Action: For discussion

Infectious Diseases

3.1.36. EMEA-002693-PIP01-19

Treatment of chronic viral hepatitis B / Treatment of chronic hepatitis B infection

Day 60 discussion

Action: For discussion

Infectious Diseases

3.1.37. Pritelivir - EMEA-002180-PIP02-19

Treatment of herpes simplex virus disease

Day 60 discussion

Action: For discussion

Infectious Diseases

3.1.38. Soticlestat - EMEA-002572-PIP02-19

Dravet syndrome / Lennox-Gastaut syndrome / Treatment of seizures associated with Dravet syndrome / Treatment of seizures associated with Lennox-Gastaut syndrome

Day 60 discussion

Action: For discussion

Neurology

3.1.39. Mosunetuzumab - EMEA-002524-PIP02-19

Treatment of mature B-cell neoplasms / Treatment of children from 6 months to less than 18 years of age with relapsed or refractory high-grade mature B-cell non-Hodgkin lymphoma (B-NHL), including Burkitt lymphoma (BL), Burkitt leukaemia (mature B-cell acute lymphoblastic leukaemia FAB L3; B-AL), and diffuse large B-cell lymphoma (DLBCL)

Day 60 discussion

Action: For discussion

Oncology

3.1.40. Recombinant anti-human CD20 and anti-human CD3 monoclonal antibody - EMEA-002648-PIP01-19

Treatment of mature B-cell neoplasms / Treatment of children from 6 months to less than 18 years of age with relapsed or refractory high-grade mature B-cell non-Hodgkin lymphoma (B-NHL), including Burkitt lymphoma (BL), Burkitt leukaemia (mature B-cell acute lymphoblastic leukaemia FAB L3; B-AL), and diffuse large B-cell lymphoma (DLBCL)

Day 60 discussion

Action: For discussion

Oncology

3.1.41. Avapritinib - Orphan - EMEA-002358-PIP03-19

Blueprint Medicines (Netherlands) B.V.; Treatment of mastocytosis / Treatment of indolent systemic mastocytosis / Treatment of smoldering systemic mastocytosis

Day 60 discussion

Action: For discussion

Oncology / Haematology-Hemostaseology

3.1.42. [Anti-neonatal Fc receptor human monoclonal antibody - Orphan - EMEA-002559-PIP03-19](#)

Momenta Pharmaceuticals, Inc.; Autoimmune haemolytic anaemia

Day 60 discussion

Action: For discussion

Other

3.1.43. [Urea / glycerol - EMEA-002511-PIP02-19](#)

Treatment of dry skin

Day 30 discussion

Action: For discussion

Dermatology

3.1.44. [Lerodalcibep - EMEA-002720-PIP01-19](#)

Treatment of elevated cholesterol / Treatment of elevated low-density lipoprotein cholesterol (LDL-C) in children from 6 to less than 18 years of age with heterozygous familial hypercholesterolaemia (HeFH) or with homozygous familial hypercholesterolaemia (HoFH)

Day 30 discussion

Action: For discussion

Endocrinology-Gynaecology-Fertility-Metabolism

3.1.45. [Recombinant human acid alpha-glucosidase - Orphan - EMEA-002447-PIP01-18](#)

Amicus Therapeutics Europe Limited; Treatment of glycogen storage disease type II (Pompe's disease)

Day 30 discussion

Action: For discussion

Endocrinology-Gynaecology-Fertility-Metabolism

3.1.46. Garadacimab - EMEA-002726-PIP01-19

Hereditary angioedema attacks (HAE)

Day 30 discussion

Action: For discussion

Haematology-Hemostaseology

3.1.47. Recombinant adeno-associated viral vector serotype 6 encoding the B-domain-deleted human factor VIII - Orphan - EMEA-002724-PIP01-19

Pfizer Europe MA EEIG; Treatment of haemophilia A (congenital FVIII deficiency)

Day 30 discussion

Action: For discussion

Haematology-Hemostaseology

3.1.48. Alpha1-proteinase inhibitor (human) - EMEA-001312-PIP03-19

Treatment of acute graft-versus-host disease (GVHD)

Day 30 discussion

Action: For discussion

Immunology-Rheumatology-Transplantation

3.1.49. Doravirine / Islatravir - EMEA-002707-PIP01-19

Treatment of human immunodeficiency virus-1 (HIV-1) infection. Doravirine/Islatravir is indicated alone or in combination with other antiretroviral medicinal products for the treatment of HIV-1 infection in paediatric patients from 28 days to less than 18 years of age

Day 30 discussion

Action: For discussion

Infectious Diseases

3.1.50. Vonoprazan - EMEA-002703-PIP01-19

Helicobacter pylori /Reflux oesophagitis / Treatment of erosive reflux oesophagitis and relief of heartburn / Eradication of helicobacter pylori (H. pylori) concurrently given with appropriate antibiotic therapy

Day 30 discussion

Action: For discussion

Infectious Diseases / Gastroenterology-Hepatology

3.1.51. Natalizumab - EMEA-001095-PIP03-19

Treatment of multiple sclerosis (MS) / Treatment of relapsing-remitting multiple sclerosis

Day 30 discussion

Action: For discussion

Neurology

3.1.52. Autologous CD4 and CD8 T-cells that have been transduced with a self-inactivating lentiviral vector expressing an affinity enhanced NY-ESO-1 specific T-Cell Receptor - Orphan - EMEA-002476-PIP02-19

GlaxoSmithKline Trading Services Limited; Soft tissue sarcoma

Day 30 discussion

Action: For discussion

Oncology

3.1.53. Efbemalenograstim alfa - EMEA-002507-PIP02-19

Prevention of chemotherapy-induced neutropenia and febrile neutropenia / Reduction in the duration of neutropenia and the incidence of febrile neutropenia in patients treated with cytotoxic chemotherapy for malignancy (with the exception of chronic myeloid leukaemia and myelodysplastic syndromes)

Day 30 discussion

Action: For discussion

Oncology

3.1.54. Lazertinib - EMEA-002725-PIP01-19

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

Day 30 discussion

Action: For discussion

Oncology

3.1.55. Relatlimab / nivolumab - EMEA-002727-PIP01-19

Treatment of melanoma / Relatlimab/nivolumab fixed dose combination for treatment of unresectable or metastatic melanoma in patients from 12 to 18 years

Day 30 discussion

Action: For discussion

Oncology

3.1.56. 4-{(2S,4S)-4-Ethoxy-1-[(5-methoxy-7-methyl-1H-indol-4-yl)methyl]piperidin-2-yl}benzoic acid hydrochloride(1/1) - Orphan - EMEA-002705-PIP01-19

Novartis Europharm Limited; C3 glomerulopathy

Day 30 discussion

Action: For discussion

Other

3.1.57. EMEA-002705-PIP02-19

IgA nephropathy

Day 30 discussion

Action: For discussion

Other

3.1.58. Alpelisib - EMEA-002016-PIP03-19

PIK3CA related overgrowth spectrum (PROS)

Day 30 discussion

Action: For discussion

Other

3.1.59. Lanadelumab - Orphan - EMEA-001864-PIP02-19

Shire Pharmaceuticals Ireland Limited (a Takeda company); Prevention of acquired angioedema attacks (AAE) / Prevention of attacks of acquired angioedema (AAE) due to C1-INH deficiency

Day 30 discussion

Action: For discussion

Other

3.1.60. Lanadelumab - Orphan - EMEA-001864-PIP03-19

Shire Pharmaceuticals Ireland Limited (a Takeda company); Prevention of attacks of idiopathic non-histaminergic angioedema (INHA)

Day 30 discussion

Action: For discussion

Other

3.1.61. [Crincerfont - EMEA-002700-PIP01-19](#)

Treatment of congenital adrenal hyperplasia

Day 30 discussion

Action: For discussion

Endocrinology-Gynaecology-Fertility-Metabolism

3.1.62. [EMEA-002714-PIP01-19](#)

Treatment of mature B-cell neoplasms

Day 30 discussion

Action: For discussion

Oncology

3.1.63. [EMEA-002674-PIP01-19](#)

Treatment of acne vulgaris

Day 30 discussion

Action: For discussion

Dermatology

3.1.64. [Lebrikizumab - EMEA-002536-PIP01-18](#)

Treatment of atopic dermatitis

Day 90 discussion

Action: For discussion

Dermatology

3.1.65. [EMEA-002619-PIP01-19](#)

Treatment of renal tumors

Day 30 discussion

Action: For discussion

Oncology

3.1.66. [Venglustat - Orphan - EMEA-001716-PIP04-19](#)

Genzyme Europe B.V.; E.75.0 GM2 gangliosidosis, E.75.1 Other gangliosidosis / GM1, GM3, E.77.1 defects in glycoprotein degradation, sialidosis / The planned indication for venglustat is long term treatment of patients with a confirmed diagnosis of late onset GM2

gangliosidosis / Long term treatment in patients within the same biochemical pathway as GM2 gangliosidosis / Long term treatment in patients with juvenile (subacute) and adolescent (late-onset) GM2 gangliosidosis ages 2 years old and older, males/females

Day 30 discussion

Action: For discussion

Endocrinology-Gynaecology-Fertility-Metabolism / Neurology

[3.1.67. Romiplostim - EMEA-000653-PIP02-19](#)

Secondary thrombocytopenia / Treatment of chemotherapy-induced thrombocytopenia (CIT) in children <18 years of age with solid tumours

Day 30 discussion

Action: For discussion

Oncology / Haematology-Hemostaseology

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. Liraglutide - EMEA-C2-000128-PIP02-09-M03](#)

Novo Nordisk A/S; Treatment of obesity

Day 30 discussion

Action: For discussion

Endocrinology-Gynaecology-Fertility-Metabolism

[3.2.2. Lubiprostone - EMEA-C-000245-PIP01-08-M06](#)

Sucampo AG; Chronic idiopathic constipation

Day 30 discussion

Action: For discussion

Gastroenterology-Hepatology

[3.2.3. Avatrombopag - EMEA-C1-001136-PIP01-11-M01](#)

Dova Pharmaceuticals Ireland Ltd.; Treatment of idiopathic thrombocytopenic purpura

Day 30 discussion

Action: For discussion

Haematology-Hemostaseology

3.2.4. Pitolisant - EMEA-C2-001176-PIP01-11-M03

Bioprojet Pharma; Treatment of narcolepsy

Day 30 discussion

Action: For discussion

Neurology

3.2.5. Palovarotene - EMEA-C1-001662-PIP01-14-M02

Ipsen Pharma; Fibrodysplasia ossificans progressiva

Day 30 discussion

Action: For discussion

Other

3.2.6. Adjupanrix: Purified antigen fractions of inactivated split virion influenza A/VietNam/1194/2004 (H5N1) like strain used (NIBRG-14) / Prepandrix: Purified antigen fractions of inactivated split virion influenza A/Indonesia/05/2005 like strain used (PR8-IBCDC-RG2) - EMEA-C-000160-PIP01-07-M05

GlaxoSmithKline Biologicals SA; Prevention of influenza infection

Day 30 discussion

Action: For discussion

Vaccines

3.3. Discussions on Modification of an Agreed Paediatric Investigation Plan

3.3.1. Tralokinumab - EMEA-001900-PIP02-17-M03

LEO Pharma A/S; Treatment of atopic dermatitis

Day 30 discussion

Action: For discussion

Dermatology

3.3.2. Mirikizumab - EMEA-002208-PIP01-17-M01

Eli Lilly and Company; Treatment of psoriasis / Treatment of Crohn's disease / Treatment of ulcerative colitis / Treatment of moderate to severely active ulcerative colitis / Treatment of moderate-to-severe plaque psoriasis / Treatment of moderate to severely active Crohn's disease

Day 30 discussion

Action: For discussion

Dermatology / Gastroenterology-Hepatology

3.3.3. Semaglutide - EMEA-001441-PIP01-13-M03

Novo Nordisk A/S; Type 2 diabetes mellitus / Treatment of diabetes mellitus type 2

Day 30 discussion

Action: For discussion

Endocrinology-Gynaecology-Fertility-Metabolism

3.3.4. Linaclotide - EMEA-000927-PIP01-10-M05

Allergan Pharmaceuticals International Limited; Functional constipation in children

Day 30 discussion

Action: For discussion

Gastroenterology-Hepatology

3.3.5. Boceprevir - EMEA-000583-PIP01-09-M08

Merck Sharp & Dohme (Europe), Inc; Treatment of chronic hepatitis C

Day 30 discussion

Action: For discussion

Infectious Diseases

3.3.6. Tenofovir disoproxil (as fumarate) - EMEA-000533-PIP01-08-M08

Gilead Sciences International Limited; Treatment of chronic viral hepatitis B / For treatment of chronic hepatitis B in paediatric patients from 2 years of age with compensated liver disease

Day 30 discussion

Action: For discussion

Infectious Diseases

3.3.7. Galcanezumab - EMEA-001860-PIP03-16-M04

Eli Lilly and Company Limited; Prevention of migraine headaches

Day 30 discussion

Action: For discussion

Neurology

3.3.8. Lasmiditan - EMEA-002166-PIP01-17-M03

Eli Lilly and Company Limited; Migraine with and without aura

Day 30 discussion

Action: For discussion

Neurology

3.3.9. Autologous peripheral blood T cells CD4 and CD8 selected and CD3 and CD28 activated transduced with retroviral vector expressing anti CD19 CD28/CD3-zeta chimeric antigen receptor and cultured - Orphan - EMEA-001862-PIP01-15-M02

Kite Pharma EU B.V.; Treatment of acute lymphoblastic leukaemia / Treatment of relapsed or refractory B-precursor acute lymphoblastic leukaemia (r/r ALL)

Day 30 discussion

Action: For discussion

Oncology

3.3.10. Axicabtagene ciloleucel - Orphan - EMEA-002010-PIP01-16-M02

Kite Pharma EU B.V.; Treatment of mature B-cell neoplasms / Treatment of children less than 18 years of age and weighing at least 6 kg with relapsed or refractory aggressive B-cell non-Hodgkin lymphoma (NHL) who are ineligible for autologous stem cell transplant

Day 30 discussion

Action: For discussion

Oncology

3.3.11. Blinatumomab - Orphan - EMEA-000574-PIP02-12-M03

Amgen Europe B.V.; Treatment of acute lymphoblastic leukaemia / Treatment of children previously untreated with high-risk first relapse of B-precursor acute lymphoblastic leukaemia.

Day 30 discussion

Action: For discussion

Oncology

3.3.12. Copanlisib - Orphan - EMEA-001757-PIP02-15-M01

Bayer AG; Treatment of all conditions included in the category of malignant neoplasms (except haematopoietic and lymphoid tissue) / Treatment of children with a relapsed or refractory neuroblastoma, Ewing sarcoma, osteosarcoma or rhabdomyosarcoma including at first relapse, in combination with chemotherapy

Day 30 discussion

Action: For discussion

Oncology

3.3.13. [Clostridium botulinum neurotoxin type A \(150 kD\), free from complexing proteins - EMEA-001039-PIP02-12-M04](#)

Merz Pharmaceuticals GmbH; Treatment of sialorrhoea / Treatment of chronic troublesome sialorrhoea associated with neurological conditions (e.g. cerebral palsy, traumatic brain injury) and/or intellectual disability in children and adolescents aged 2 – 17 years

Day 30 discussion

Action: For discussion

Ophthalmology / Neurology

3.3.14. [Palovarotene - Orphan - EMEA-001662-PIP01-14-M03](#)

Ipsen Pharma; Treatment of fibrodysplasia ossificans progressiva

Day 30 discussion

Action: For discussion

Other

3.3.15. [Ad26.ZEBOV - EMEA-002307-PIP01-17-M01](#)

Janssen Cilag International NV; Prevention of ebola virus disease (EVD) / Prevention of EVD in children aged ≥ 1 year

Day 30 discussion

Action: For discussion

Vaccines

3.3.16. [MVA-BN-Filo - EMEA-002308-PIP01-17-M01](#)

Janssen Cilag International NV; Prevention of ebola virus disease (EVD) / Prevention of EVD in children aged ≥ 1 year

Day 30 discussion

Action: For discussion

Vaccines

3.3.17. [Regorafenib - EMEA-001178-PIP01-11-M05](#)

Bayer AG; Treatment of all conditions contained in the category of malignant neoplasms (except haematopoietic and lymphoid tissue) / Treatment of paediatric patients with a solid malignant tumour(s) integrated with anti-cancer therapy

Day 30 discussion

Action: For discussion

Oncology

3.3.18. Etrolizumab - EMEA-001434-PIP01-13-M03

Roche Registration GmbH; Treatment of ulcerative colitis / Treatment of Crohn's disease / Treatment of moderately to severely active Crohn's disease / Treatment of moderately to severely active ulcerative colitis

Day 30 discussion

Action: For discussion

Gastroenterology-Hepatology

3.3.19. Elafibranor - EMEA-001857-PIP01-15-M01

GENFIT SA; Non-alcoholic fatty liver disease (NAFLD) including non-alcoholic steatohepatitis (NASH)

Day 30 discussion

Action: For discussion

Gastroenterology-Hepatology

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 02 March 2020 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. Pentosan polysulfate sodium - EMEA-18-2019

Paradigm Biopharmaceuticals (Ireland) Ltd; Treatment primary and secondary osteoarthritis/ treatment of osteoarthritis of the knee associated with pain and bone marrow lesions

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. Macitentan - EMEA-001032-PIP01-10

Treatment of pulmonary arterial hypertension (PAH)

Proposed indication: Treatment of portopulmonary hypertension (POPH)

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

No items

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

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. Scientific Advice Working Party: nominations of PDCO members to SAWP

PDCO member: Karl-Heinz Huemer, Kirstine Moll Harboe, Johanna Wernsperger, Dina Apele, Dana Marin

Action: For discussion

9.3.4. Meeting Summary from the Annual Patients and Consumers Working Party (PCWP)/ Healthcare Professionals Working Party (HCPWP) with all eligible organisations - 20 November 2019

Action: For information

9.3.5. Agenda for the Patients and Consumers Working Party (PCWP)/ Healthcare Professionals Working Party (HCPWP) on 03-04 March 2020

Action: For information

9.4. Cooperation within the EU regulatory network

9.4.1. CTFG: Outcome of TC

CTFG Chair: Ann Marie Janson Lang;

Action: For information

9.5. Cooperation with International Regulators

9.5.1. Report from the Paediatric Cluster Teleconference

Action: For information

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

No items

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 Tuesday, 19:00 - 20:00, room 2A

11.1.2. Neonatology

Action: For discussion on Wednesday, 07:30 - 08:30, room 2B

11.1.3. Inventory

Action: For discussion on Tuesday, 19:00 - 20:00, room 2B

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/