29 March 2019
EMA/PDCO/161218/2019 Corr.1
Inspections, Human Medicines Pharmacovigilance and Committees Division

Paediatric Committee (PDCO)

Draft agenda for the meeting on 26-29 March 2019

Chair: Dirk Mentzer – Vice-Chair: Koenraad Norga

26 March 2019, 14:00- 17:00, room 2D

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

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

29 March 2019, 08:30- 13:00, 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 26-29 March 2019. See 26-29 March 2019 PDCO minutes (to be published post 23-26 April 2019 PDCO meeting).

1.2. Adoption of agenda

PDCO agenda for 26-29 March 2019

1.3. Adoption of the minutes

PDCO minutes for 26 February - 01 March 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. Efpeglenatide - EMEA-001903-PIP01-15

Type 2 diabetes mellitus

Day 120 opinion

Action: For adoption

Endocrinology-Gynaecology-Fertility-Metabolism

2.1.2. Dusquetide - EMEA-002306-PIP02-18

Prevention of severe oral mucositis

Day 120 opinion

Action: For adoption

Gastroenterology-Hepatology

2.1.3. Synthetic double-stranded siRNA oligonucleotide directed against antithrombin mRNA and covalently linked to a ligand containing three N-acetylgalactosamine residues - Orphan - EMEA-001855-PIP01-15

Genzyme Europe B.V.; Treatment of Haemophilia B, Treatment of Heamophilia A / Indicated for routine prophylaxis to prevent or reduce the frequency of bleeding episodes in children aged ≥ 1 year with severe Haemophilia B, including patients who express neutralizing antibodies to exogenous factor IX substitution /Indicated for routine prophylaxis to prevent or reduce the frequency of bleeding episodes in children aged ≥ 1 year with severe Haemophilia A, including patients who express neutralizing antibodies to exogenous factor VIII substitution

Day 120 opinion

Action: For adoption

Haematology-Hemostaseology

2.1.4. Ustekinumab - EMEA-000311-PIP06-18

ICD10: M32 Treatment of systemic lupus erythematosus (SLE)

Day 120 opinion

Action: For adoption

Immunology-Rheumatology-Transplantation

2.1.5. Tafenoquine - EMEA-002301-PIP01-17

Prevention of malaria

Day 120 opinion

Action: For adoption

Infectious Diseases

2.1.6. Benzimidazole-containing ENaC inhibitor - EMEA-002394-PIP01-18

Treatment of cystic fibrosis (CF) / Indicated to improve lung function and reduce pulmonary exacerbations for patients with CF in conjunction with standard therapies

Day 120 opinion

Action: For adoption

Pneumology - Allergology

2.1.7. Glycopyrronium bromide / formoterol fumarate dihydrate / beclometasone dipropionate - EMEA-001875-PIP02-18

Treatment of asthma / Regular treatment of asthma in patients not controlled with medium-high doses of inhaled corticosteroids and long-acting beta2-agonists

Day 120 opinion

Action: For adoption

Pneumology - Allergology

2.1.8. Atorvastatin / amlodipine / candesartan - EMEA-002520-PIP01-18

Treatment of essential hypertension (ICD9: 401, ICD10: I10) / Treatment of familial hypercholesterolemia (ICD9: 272.0, ICD10: E78.0) / For adults with hypertension and elevated cholesterol already controlled with ramipril, amlodipine and atorvastatin given concurrently at the same dose level as in the fixed dose combination (FDC), (substitution indication)

Day 60 opinion

Action: For adoption

Cardiovascular Diseases

2.1.9. Recombinant human lecithin cholesterol acyltransferase - Orphan - EMEA-002497-PIP01-18

AstraZeneca AB; Acute ST-segment elevation myocardial infarction (STEMI)

Day 60 opinion

Action: For adoption

Cardiovascular Diseases

2.1.10. Inactivated patient's own (autologous) microorganism (e.g. Escherichia coli, Candida spp., Enterococcus spp., Streptococcus spp., Staphylococcus spp., Prevotella intermedia, Fusobacterium nucleatum and others) - EMEA-002442-PIP01-18

Prevention and treatment of chronic or recurrent dermal or mucosal inflammation / Prevention and treatment of chronic or recurrent skin and/or mucosa inflammation in the urogenital, otorhinolaryngeal, bronchial, oral, gingiva or periodontal tract, resistant to treatment or not sufficiently treatable with topical or systemic antibiotics, antivirals, antifungals or anti-inflammatory compounds

Day 60 opinion

Action: For adoption

Dermatology / Pneumology - Allergology / Oto-rhino-laryngology / Uro-nephrology

2.1.11. Seladelpar - Orphan - EMEA-002527-PIP01-18

CymaBay Ireland Limited; Treatment of primary biliary cholangitis

Day 60 opinion

Action: For adoption

Gastroenterology-Hepatology

2.1.12. Humanized antibody targeting B cell maturation antigen conjugated with maleimidocaproyl monomethyl auristatin F - Orphan - EMEA-002468-PIP02-18

GlaxoSmithKline Trading Services; Treatment of multiple myeloma

Day 60 opinion

Action: For adoption

Oncology

2.1.13. Niraparib - Orphan - EMEA-002268-PIP03-18

Janssen Research & Development; Treatment of prostate malignant neoplasms

Day 60 opinion

Action: For adoption

Oncology

2.1.14. Carfilzomib - Orphan - EMEA-001806-PIP03-18

Amgen Europe BV; Treatment of multiple myeloma

Day 60 opinion

Action: For adoption

Oncology / Haematology-Hemostaseology

2.1.15. Amoxicillin - EMEA-002548-PIP01-19

Adults / Combination therapy for the eradication of Helicobacter pylori in patients with peptic ulceration, with the aim of reducing the frequency of recurrence of duodenal ulcer disease (ulcera duodeni) and gastric ulcer (ulcer ventriculi) caused by this pathogen

Day 60 opinion

Action: For adoption

Other

2.1.16. Clarithromycin - EMEA-002549-PIP01-19

Adults / Combination therapy for the eradication of Helicobacter pylori in patients with peptic ulceration, with the aim of reducing the frequency of recurrence of duodenal ulcer disease (ulcera duodeni) and gastric ulcer (ulcer ventriculi) caused by this pathogen.

Day 60 opinion

Action: For adoption

Other

2.1.17. Ibandronic acid - EMEA-002331-PIP01-18

Treatment of osteoporosis

Day 60 opinion

Action: For adoption

Other

2.1.18. Colecalciferol - EMEA-002553-PIP01-19

Treatment of osteoporosis

Day 60 opinion

Action: For adoption

2.1.19. Pantoprazole - EMEA-002512-PIP01-18

Adults / Combination therapy for the eradication of Helicobacter pylori in patients with peptic ulceration, with the aim of reducing the frequency of recurrence of duodenal ulcer disease (ulcera duodeni) and gastric ulcer (ulcer ventriculi) caused by this pathogen.

Day 60 opinion

Action: For adoption

Other

2.1.20. EMEA-002398-PIP01-18[1]

Cystic Fibrosis / Treatment of cystic fibrosis in individuals with cystic fibrosis who are homozygous for the F508del mutation and are receiving treatment with a CFTR modulator

Day 120 opinion

Action: For adoption

Pneumology – Allergology

[1] Addition in corrigendum 1

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. Osilodrostat - EMEA-C2-000315-PIP02-15-M02

Novartis Europharm Limited; Treatment of adrenal cortical hyperfunctions

Day 60 letter

Action: For adoption

Endocrinology-Gynaecology-Fertility-Metabolism

2.2.2. Lurasidone hydrochloride - EMEA-C-001230-PIP01-11-M04

Aziende Chimiche Riunite Angelini Francesco - ACRAF S.p.A; Treatment of schizophrenia

Day 60 opinion

Action: For adoption

Psychiatry

2.2.3. Potassium hydrogen carbonate / potassium citrate monohydrated - EMEA-C1-001357-PIP01-12-M02

ADVICENNE; Treatment of renal tubular acidosis

Day 60 letter

Action: For adoption

Uro-nephrology

2.3. Opinions on Modification of an Agreed Paediatric Investigation Plan

2.3.1. Angiotensin II - EMEA-001912-PIP02-16-M02

La Jolla Pharmaceutical II B.V.; Hypotension associated with distributive or vasodilatory shock

Day 60 opinion

Action: For adoption

Cardiovascular Diseases

2.3.2. Omecamtiv mecarbil - EMEA-001696-PIP01-14-M01

Amgen Europe B.V.; Treatment of heart failure / Treatment of chronic heart failure New York Association (NYHA) class II-IV with systolic dysfunction, in children and adolescents 6

to <18 years, in combination with standard pharmacological therapy, including angiotensin converting enzyme inhibitors (ACE inhibitors), angiotensin II receptor blockers, and/or beta-blockers

Day 60 opinion

Action: For adoption

Cardiovascular Diseases

2.3.3. Asfotase alfa - Orphan - EMEA-000987-PIP01-10-M04

Alexion Europe SAS; Treatment of hypophosphatasia

Day 60 opinion

Action: For adoption

Endocrinology-Gynaecology-Fertility-Metabolism

2.3.4. Deferiprone - Orphan - EMEA-001126-PIP01-10-M03

Consorzio per Valutazioni Biologiche e Farmacologiche (CVBF) - Coordinator for DEEP Project (HEALTH-F4-2010-261483); Treatment of chronic iron overload requiring chelation therapy / Treatment of iron overload in paediatric patients affected by haemoglobinopathies requiring chronic transfusions and iron chelation

Day 60 opinion

Action: For adoption

Haematology-Hemostaseology

2.3.5. Ravulizumab - Orphan - EMEA-002077-PIP01-16-M02

Alexion Europe SAS; Treatment of paroxysmal nocturnal haemoglobinuria

Day 60 opinion

Action: For adoption

Haematology-Hemostaseology

2.3.6. Baricitinib - EMEA-001220-PIP01-11-M05

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

Day 60 opinion

Action: For adoption

Immunology-Rheumatology-Transplantation

2.3.7. Adalimumab - EMEA-000366-PIP02-09-M06

AbbVie Limited; Ulcerative Colitis / Treatment of moderate to severe ulcerative colitis

Day 60 opinion

Action: For adoption

Immunology-Rheumatology-Transplantation / Ophthalmology / Dermatology / Gastroenterology-Hepatology

2.3.8. Imipenem monohydrate / relebactam monohydrate / cilastatin sodium - EMEA-001809-PIP01-15-M01

Merck Sharp & Dohme (Europe), Inc.; Treatment of bacterial infections caused by gram-negative bacteria

Day 60 opinion

Action: For adoption

Infectious Diseases

2.3.9. Onasemnogene abeparvovec - Orphan - EMEA-002168-PIP01-17-M01

AveXis Netherlands B.V.; Treatment of spinal muscular atrophy / Treatment of spinal muscular atrophy Type 1

Day 60 opinion

Action: For adoption

Neurology

2.3.10. Quizartinib - Orphan - EMEA-001821-PIP01-15-M03

Daiichi Sankyo Europe GmbH; Treatment of acute myeloid leukaemia (AML) / For the treatment of paediatric patients aged from 1 month to less than 18 years of age with newly diagnosed AML with FLT3-ITD mutations / For the treatment of paediatric patients aged from 1 month to less than 18 years of age with refractory or relapsed AML with FLT3-ITD mutations after failure of front line intensive chemotherapy regimen, in combination with standard chemotherapy.

Day 60 opinion

Action: For adoption

Oncology

2.3.11. Ruxolitinib phosphate - EMEA-000901-PIP03-16-M01

Novartis Europharm Limited; Treatment of acute graft versus host disease (GvHD) after allogeneic hematopoietic stem cell transplantation (alloSCT) in paediatric patients aged 28 days and above

Day 60 opinion

Action: For adoption

Oncology

2.3.12. Gilteritinib (as fumarate) - Orphan - EMEA-002064-PIP01-16-M01

Astellas Pharma Europe B.V.; Treatment of acute myeloid leukemia / Treatment of patients from 6 months to less than 18 years of age with relapsed or refractory FLT3/ITD positive acute myeloid leukaemia or newly-diagnosed FLT3/ITD positive acute myeloid leukaemia

Day 60 opinion

Action: For adoption

Oncology / Haematology-Hemostaseology

2.3.13. Clostridium botulinum neurotoxin type A (150 kD), free from complexing proteins - EMEA-001039-PIP02-12-M03

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

Day 60 opinion

Action: For adoption

Ophthalmology / Neurology

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

Les Laboratoires Servier; Treatment of major depressive episodes

Day 60 opinion

Action: For adoption

Psychiatry

2.3.15. Finerenone - EMEA-001623-PIP01-14-M02

Bayer AG; Treatment of chronic kidney disease associated with proteinuria in addition to a therapy with angiotensin converting enzyme inhibitor (ACEi) or angiotensin-receptor blocker (ARB)

Day 60 opinion

Action: For adoption

Uro-nephrology

2.4. Opinions on Re-examinations

2.4.1. Chemically modified recombinant human sulfamidase - Orphan - EMEA-002380-PIP01-18

Swedish Orphan Biovitrum AB (publ); Mucopolysaccharidosis type IIIA (MPS IIIA)

Day 30 opinion

Action: For adoption

Endocrinology-Gynaecology-Fertility-Metabolism

2.5. Opinions on Review of Granted Waivers

No items

2.6. Finalisation and adoption of opinions

2.7. Partial Compliance Checks completed by EMA

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

2.7.1. B (Victoria lineage)/A (H1N1)/B (Yamagata lineage)/A (H3N2) - EMEA-C2-001715-PIP01-14-M02

Seqirus Netherlands B.V.; Prevention of influenza / Active immunisation against influenza in children of 6 to less than 72 months of age / Active immunisation of influenza in elderly subjects of 65 years and older

Day 1 letter

Action: For information

Vaccines

2.7.2. Cobicistat - EMEA-C1-000969-PIP01-10-M04

Gilead Sciences Ireland UC; Treatment of human immunodeficiency virus type-1 (HIV-1) infection.

Day 1 letter

Action: For information

Infectious Diseases

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. Dihomo-γ-linolenic acid (DGLA) - EMEA-002364-PIP01-18

Treatment of moderate to severe atopic dermatitis

Day 90 discussion

Action: For discussion

Dermatology

3.1.2. Adeno-associated viral vector serotype 5 containing a B domain deleted variant of human coagulation factor VIII gene - Orphan - EMEA-002427-PIP01-18

BioMarin International Limited; Treatment of patients with haemophilia A

Day 90 discussion

Action: For discussion

Haematology-Hemostaseology

3.1.3. Dexamethasone - EMEA-002423-PIP01-18

ICD10 H59.9 Post-procedural disorder of eye and adnexa

Day 90 discussion

Action: For discussion

Ophthalmology

3.1.4. Lentiviral vector containing the human ABCA4 gene for treatment of Stargardt's disease - Orphan - EMEA-002407-PIP01-18

Sanofi-Aventis Recherche & Développement; Treatment of inherited retinal disorders

Day 90 discussion

Action: For discussion

Ophthalmology

3.1.5. Glycerol / urea - EMEA-002511-PIP01-18

Treatment of atopic dermatitis / Treatment of dry skin / Prevention of relapse of atopic dermatitis

Day 60 discussion

Action: For discussion

Dermatology

3.1.6. EMEA-002501-PIP01-18

Treatment and prophylaxis of bleeding in patients with haemophilia A (congenital FVIII deficiency)

Day 60 discussion

Action: For discussion

Haematology-Hemostaseology

3.1.7. EMEA-002529-PIP01-18

Treatment of respiratory syncytial virus infection

Day 60 discussion

Action: For discussion

Infectious Diseases

3.1.8. Cladribine - EMEA-000383-PIP02-18

Treatment of multiple sclerosis / Adults and Paediatrics

Day 60 discussion

Action: For discussion

Neurology

3.1.9. Phenobarbital - EMEA-002532-PIP01-18

Epilepsy

Day 60 discussion

Action: For discussion

Neurology

3.1.10. Abemaciclib - EMEA-002342-PIP02-18

High grade glioma (HGG), Neuroblastoma (NBL) / Treatment of relapsed or refractory

neuroblastoma in combination with irinotecan and temozolomide in pediatric patients / Treatment of newly diagnosed high grade glioma in combination with temozolomide in pediatric patients

Day 60 discussion

Action: For discussion

Oncology

3.1.11. Olopatadine hydrochloride / mometasone furoate - EMEA-002514-PIP01-18

Treatment of allergic rhinitis / rhino-conjunctivitis

Day 60 discussion

Action: For discussion

Oto-rhino-laryngology

3.1.12. Atogepant - EMEA-002530-PIP01-18

G43 Migraine / Prophylaxis of migraine

Day 60 discussion

Action: For discussion

Pain

3.1.13. (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 60 discussion

Action: For discussion

Pneumology - Allergology

3.1.14. Budesonide / salbutamol sulfate - EMEA-002533-PIP01-18

Treatment of asthma / as-needed treatment or prevention of bronchoconstriction in children aged 6 years and older with reversible obstructive airway disease. The reduction of exacerbations in children aged 6 years and older with asthma

Day 60 discussion

Action: For discussion

Pneumology - Allergology

3.1.15. Tosatoxumab - Orphan - EMEA-002506-PIP01-18

Aridis Pharmaceuticals Inc; Pneumonia caused by Staphylococcus aureus

Day 60 discussion

Action: For discussion

Pneumology - Allergology

3.1.16. Ramipril / bisoprolol - EMEA-002531-PIP01-18

Treatment of chronic (systolic) heart failure (ICD10: I50.22) / Treatment of coronary artery disease (ICD10: I25-1), Treatment of essential hypertension (ICD10: I10) / Treatment of hypertension with stable coronary artery disease and those with stable chronic heart failure with reduced systolic left ventricular function in adult patients adequately controlled with bisoprolol and ramipril given concurrently at the same dose level (substitution indication)

Day 30 discussion

Action: For discussion

Cardiovascular Diseases

3.1.17. Genetically modified replication-incompetent herpes simplex virus-1 expressing collagen VII - Orphan - EMEA-002472-PIP02-19

Krystal Biotech, Inc.; Dystrophic epidermolysis bullosa

Day 30 discussion

Action: For discussion

Dermatology

3.1.18. Human monoclonal IgG2 antibody against tissue factor pathway inhibitor - Orphan - EMEA-002498-PIP01-18

Bayer AG; Treatment of haemophilia A / Treatment of haemophilia B

Day 30 discussion

Action: For discussion

Haematology-Hemostaseology

3.1.19. Sutimlimab - Orphan - EMEA-002542-PIP01-18

Bioverativ Inc; Treatment of primary cold agglutinin disease

Day 30 discussion

Action: For discussion

Haematology-Hemostaseology

3.1.20. EMEA-002528-PIP01-19

Chronic idiopathic arthritis / Treatment of juvenile idiopathic arthritis

Day 30 discussion

Action: For discussion

Immunology-Rheumatology-Transplantation

3.1.21. Ibrexafungerp citrate - EMEA-002535-PIP01-18

Vulvovaginal candidiasis

Day 30 discussion

Action: For discussion

Infectious Diseases

3.1.22. Iclaprim mesylate - EMEA-002391-PIP02-19

Infection with gram-positive bacteria

Day 30 discussion

Action: For discussion

Infectious Diseases

3.1.23. 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 30 discussion

Action: For discussion

Oncology

3.1.24. Abemaciclib - EMEA-002342-PIP03-18

Treatment of breast cancer

Day 30 discussion

Action: For discussion

Oncology

3.1.25. Capivasertib - EMEA-002551-PIP01-18

Prostate cancer / Breast cancer

Day 30 discussion

Action: For discussion

Oncology

3.1.26. Tisotumab vedotin - EMEA-002522-PIP01-18

Treatment of cervical cancer

Day 30 discussion

Action: For discussion

Oncology

3.1.27. Trilaciclib - EMEA-002534-PIP02-19

Prevention of chemotherapy induced myelosuppression

Day 30 discussion

Action: For discussion

Oncology

3.1.28. EMEA-002503-PIP01-18

Biochemical recurrence of prostate cancer

Day 30 discussion

Action: For discussion

Oncology / Uro-nephrology

3.1.29. Emiplacel - EMEA-002539-PIP01-18

Treatment of peripheral ischaemia

Day 30 discussion

Action: For discussion

Other

3.1.30. Human immunoglobulin G2 isotype antibody to IL-33R - EMEA-002515-PIP01-18

Treatment of asthma / Add-on therapy for the maintenance treatment for moderate-severe asthma

Day 30 discussion

Action: For discussion

Pneumology - Allergology

3.1.31. EMEA-002519-PIP02-18

Treatment of schizophrenia

Day 30 discussion

Action: For discussion

Psychiatry

3.1.32. Perampanel- EMEA-000467-PIP01-08-M11

Treatment of treatment-resistant epilepsies

Day 30 discussion

Action: For discussion

Neurology

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. Emicizumab - EMEA-C-001839-PIP01-15

Roche Registration GmbH; Treatment of hereditary FVIII deficiency

Day 30 discussion

Action: For discussion

Haematology-Hemostaseology

3.2.2. Turoctocog alfa - EMEA-C-000428-PIP01-08-M03

Novo Nordisk A/S; Treatment of hereditary Factor VIII deficiency

Day 30 discussion

Action: For discussion

Haematology-Hemostaseology

3.2.3. Cobicistat / darunavir - EMEA-C3-001280-PIP01-12-M02

Janssen-Cilag International NV; Treatment of human immunodeficiency virus type-1 (HIV-1) infection

Day 30 discussion

Action: For discussion

Infectious Diseases

3.2.4. Split influenza virus, inactivated containing antigen equivalent to A/ California/7/2009(H1N1)-like strain (A/California/7/2009), adjuvanted - EMEA-C-000669-PIP01-09-M02

Sanofi Pasteur SA; Influenza

Day 30 discussion

Action: For discussion

Vaccines

3.3. Discussions on Modification of an Agreed Paediatric Investigation Plan

3.3.1. Semaglutide - EMEA-001441-PIP02-15-M02

Novo Nordisk; Type 2 Diabetes Mellitus

Day 30 discussion

Action: For discussion

Endocrinology-Gynaecology-Fertility-Metabolism

3.3.2. Obeticholic Acid - Orphan - EMEA-001304-PIP02-13-M04

Intercept Pharma Ltd.; Primary biliary cholangitis (PBC) / Biliary atresia

Day 30 discussion

Action: For discussion

Gastroenterology-Hepatology

3.3.3. Potassium chloride / sodium chloride / ascorbic acid / sodium ascorbate / sodium sulfate / Ppolyethylene glycol 3350 - EMEA-001705-PIP02-15-M02

Norgine Limited; Bowel cleansing prior to clinical procedures

Day 30 discussion

Action: For discussion

Gastroenterology-Hepatology

3.3.4. Rilpivirine - EMEA-000317-PIP01-08-M11

Janssen-Cilag International NV; Treatment of human immunodeficiency virus type-1 (HIV-1) infection / rilpivirine is indicated in combination with other antiretroviral (ARV) medicinal products, for the treatment of human immunodeficiency virus type-1 (HIV-1) infection in ARV-naïve paediatric patients from 2 to less than 18 years with a baseline viral load below 100,000 HIV-1 RNA copies/mL.

Day 30 discussion

Action: For discussion

Infectious Diseases

3.3.5. 2-Iminobiotin - Orphan - EMEA-001070-PIP01-10-M01

Neurophyxia B.V.; Treatment of perinatal asphyxia

Day 30 discussion

Action: For discussion

Neonatology - Paediatric Intensive Care

3.3.6. Atezolizumab - EMEA-001638-PIP01-14-M02

Roche Registration GmbH; 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 birth to less than 18 years old with a PD-L1 positive paediatric solid tumour as part of the first line treatment

Day 30 discussion

Action: For discussion

Oncology

3.3.7. Autologous CD4+ and CD8+ T cells Expressing a CD19-Specific Chimeric Antigen Receptor (lisocabtagene maraleucel) - Orphan - EMEA-001995-PIP01-16-M02

Celgene Europe B.V.; Treatment of B-lymphoblastic leukemia/lymphoma, Treatment of mature B-cell neoplasms / Treatment of paediatric patients with CD19+ relapsed or refractory B-cell acute lymphoblastic leukaemia / Treatment of paediatric patients with CD19+ relapsed or refractory diffuse-large B-cell lymphoma, burkitt lymphoma or primary mediastinal large B-cell lymphoma

Day 30 discussion

Action: For discussion

Oncology

3.3.8. Daratumumab - Orphan - EMEA-002152-PIP01-17-M01

Janssen-Cilag International NV; Treatment of lymphoid malignancies (except mature B-cell

neoplasms) / Treatment of paediatric patients from birth to less than 18 years of age with a lymphoid malignancy (except mature B-cell neoplasms)

Day 30 discussion

Action: For discussion

Oncology

3.3.9. Ibrutinib - Orphan - EMEA-001397-PIP03-14-M04

Janssen-Cilag International N.V.; Treatment of mature B-cell neoplasm / Treatment of children from 1 year to less than 18 years of age with newly-diagnosed and relapsed/refractory mature B-cell lymphoma, that is, diffuse large B-cell lymphoma or burkitt and burkitt-like lymphoma.

Day 30 discussion

Action: For discussion

Oncology

3.3.10. Isatuximab - Orphan - EMEA-002205-PIP01-17-M01

Sanofi-Aventis Recherche & Développement; Treatment of malignant neoplasms of the haematopoietic and lymphoid tissue / Treatment of relapsed, refractory and newly-diagnosed acute lymphoblastic leukemia in combination with standard treatment in paediatric patients from 28 days to less than 18 years of age, Treatment of relapsed, refractory and newly-diagnosed acute myeloid leukemia in combination with standard treatment in paediatric patients from 28 days to less than 18 years of age

Day 30 discussion

Action: For discussion

Oncology

3.3.11. Lenvatinib - EMEA-001119-PIP02-12-M05

Eisai Europe Ltd; Treatment of papillary thyroid carcinoma / Treatment of osteosarcoma, Treatment of follicular thyroid carcinoma / Treatment of refractory or relapsed osteosarcoma in children and adolescents / Treatment of progressive, radioiodine-refractory differentiated thyroid cancer in children and adolescents

Day 30 discussion

Action: For discussion

Oncology

3.3.12. Palbociclib - EMEA-002146-PIP01-17-M01

Pfizer Europe MA EEIG; Treatment of refractory or recurrent Ewing sarcoma

Day 30 discussion

Action: For discussion

Oncology

3.3.13. Autologous CD34+ enriched cell fraction that contains CD34+ cells transduced with lentiviral vector that encodes for the human ARSA cDNA sequence - Orphan - EMEA-001765-PIP02-15-M03

Orchard Therapeutics (Europe) Ltd; Treatment of metachromatic leukodystrophy (MLD)

Day 30 discussion

Action: For discussion

Other

3.3.14. Allogeneic T cells genetically modified with a retroviral vector encoding for a truncated form of the human low affinity nerve growth factor receptor (ΔLNGFR) and the herpes simplex I virus thymidine kinase (HSV-TK Mut2) - Orphan - EMEA-001370-PIP02-13-M02

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

Day 30 discussion

Action: For discussion

Other / Haematology-Hemostaseology

3.3.15. Aqueous extracts of Dermatophagoides pteronyssinus and Dermatophagoides farinae - EMEA-000815-PIP01-09-M01

Allergy Therapeutics (UK) Ltd; Allergic rhinitis and acute atopic conjunctivitis due to house dust mites / allergic rhinitis/allergic conjunctivitis

Day 30 discussion

Action: For discussion

Pneumology - Allergology

3.3.16. Birch, Hazel and Alder Pollen Extract - EMEA-000808-PIP01-09-M01

Allergy Therapeutics (UK) Ltd; J.30.1 Allergic rhinitis due to pollen H10.1 Acute atopic conjunctivitis / Allergic rhinitis/allergic conjunctivitis

Day 30 discussion

Action: For discussion

Pneumology - Allergology

3.3.17. Vortioxetine - EMEA-000455-PIP02-10-M05

H. Lundbeck A/S; Major Depressive Disorder

Day 30 discussion

Action: For discussion

Psychiatry

3.3.18. Fc- and CDR-modified humanized monoclonal antibody against C5 - Orphan - EMEA-001943-PIP01-16-M02

Alexion Europe SAS; Treatment of atypical haemolytic uremic syndrome

Day 30 discussion

Action: For discussion

Uro-nephrology / Haematology-Hemostaseology

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 28 May 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

Disclosure of 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

Disclosure of 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)

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

6. Discussion on the applicability of class waivers

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

6.1. Discussions on the applicability of class waiver for products

6.1.1. Cetuximab-IRDye 700DX Conjugate - EMEA-01-2019

Rakuten Aspyrian, Inc.; The class of Her- / Epidermal growth factor-receptor antibody medicinal products for treatment of head and neck epithelial malignant neoplasms and the class of photosensitising medicinal products for treatment of head and neck epithelial malignant neoplasms/ Treatment, in combination with a PIT690 Laser System, of patients with locoregional recurrent head and neck squamous cell carcinoma (HNSCC) with progressive disease on or after two or more lines of therapy

Action: For adoption

6.1.2. Leuco-methylthioninium bis(hydromethanesulfonate) - EMEA-04-2019

TauRx Therapeutics Europe Ltd.; All classes of medicinal products for treatment of Alzheimer’s disease / Treatment of early/mild/moderate Alzheimer's Disease

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

No items

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

Update on 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. 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

9.4. Cooperation within the EU regulatory network

9.4.1. European Network of Paediatric Research (Enpr) - European Medicines Agency (EMA): Introduction to activities of PEDMED-NL

Action: For information

9.4.2. European Network of Paediatric Research (Enpr) - European Medicines Agency (EMA): Update on outcome of Working Group on Trial Preparedness

PDCO member: Angeliki Siapkara

Action: For information

9.4.3. Handling of confidential information within the EU network

Action: For discussion

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

9.8.1. Strategic Review and Learning Meeting (SRLM) under the Romanian Presidency to be held in Malta on 13-14 June 2019

PDCO members: Dana Gabriela Marin, John Joseph Borg

Action: For information

10. Any other business

No items

11. Breakout sessions

11.1.1. Paediatric oncology

Action: For discussion on Thursday, 12:30 - 13:30, room 0-E

11.1.2. Neonatology

Action: For discussion on Thursday, 12:30 - 13:30, room 0-F

11.1.3. Inventory

Action: For discussion on Thursday, 12:30 - 13:30, room 1-A

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/