9 November 2020
EMA/PDCO/557670/2020
Human Medicines Division

Paediatric Committee (PDCO)

Agenda for the meeting on 10-13 November 2020

Chair: Koenraad Norga – Vice-Chair: Sabine Scherer

10 November 2020, 14:00- 19:00, Virtual meeting

11 November 2020, 08:30- 19:00, Virtual meeting

12 November 2020, 08:30- 19:00, Virtual meeting

13 November 2020, 08:30- 13:00, Virtual meeting

Health and safety information

In accordance with the Agency's health and safety policy, delegates are to be briefed on health, safety and emergency information and procedures prior to the start of the meeting.

Disclaimers

Some of the information contained in this agenda is considered commercially confidential or sensitive and therefore not disclosed. With regard to intended therapeutic indications or procedure scopes listed against products, it must be noted that these may not reflect the full wording proposed by applicants and may also vary during the course of the review. Additional details on some of these procedures will be published in the PDCO Committee meeting reports (after the PDCO Opinion is adopted), and on the Opinions and decisions on paediatric investigation plans webpage (after the EMA Decision is issued).

Note on access to documents

Some documents mentioned in the agenda cannot be released at present following a request for access to documents within the framework of Regulation (EC) No 1049/2001 as they are subject to on-going procedures for which a final decision has not yet been adopted. They will become public when adopted or considered public according to the principles stated in the Agency policy on access to documents (EMA/127362/2006).

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Table of contents

1. Introductions

1.1. Welcome and declarations of interest of members, alternates and experts

Pre-meeting list of participants and restrictions in relation to declarations of interests applicable to the items of the agenda for the PDCO plenary session to be held 10-13 November 2020. See 10-13 November 2020 PDCO minutes (to be published post 8-11 December 2020 PDCO meeting).

1.2. Adoption of agenda

PDCO agenda for 10-13 November 2020

1.3. Adoption of the minutes

PDCO minutes for 13-16 October 2020

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. Venglustat - Orphan - EMEA-001716-PIP05-20

Genzyme Europe B.V.; Treatment of autosomal dominant polycystic kidney disease/ Indicated for long term treatment to slow the progression of cysts development in paediatric patients from 12 years to <18 years old with autosomal dominant polycystic kidney disease

Day 120 opinion

Action: For adoption

Endocrinology-Gynaecology-Fertility-Metabolism / Uro-nephrology

2.1.2. Etrasimod L-arginine - EMEA-002713-PIP01-19

Treatment of ulcerative colitis / Treatment of moderately or severely active ulcerative colitis

Day 120 opinion

Action: For adoption

Gastroenterology-Hepatology

2.1.3. Etranacogene dezaparvovec - Orphan - EMEA-002722-PIP01-19

uniQure biopharma B.V.; Treatment of Haemophilia B

Day 120 opinion

Action: For adoption

Haematology-Hemostaseology

2.1.4. Olinciguat - EMEA-002759-PIP01-19

Treatment of sickle cell disease (SCD)

Day 120 opinion

Action: For adoption

Haematology-Hemostaseology

2.1.5. Voxelotor - Orphan - EMEA-002356-PIP02-20

Synteract GmbH; sickle cell disease

Day 120 opinion

Action: For adoption

Haematology-Hemostaseology

2.1.6. EMEA-002742-PIP01-19

Immunocompromised patients due to having received a solid organ transplant or recent Hematopoietic Stem Cell Transplantation or chemotherapy are high risk of hospitalized patients who are infected with Parainfluenza viral pneumonia and there is no authorised medicinal product for Parainfluenza infection

Day 120 opinion

Action: For adoption

Infectious Diseases

Note: Withdrawal request received on 05/11/2020

2.1.7. EMEA-002755-PIP01-19

Prevention of lower respiratory tract infection caused by respiratory syncytial virus

Day 120 opinion

Action: For adoption

Infectious Diseases

2.1.8. Sulbactam / durlobactam - EMEA-002807-PIP01-20

Treatment of infections due to organisms of the Acinetobacter baumannii-calcoaceticus complex / Treatment of infections due to Acinetobacter baumannii-calcoaceticus complex in patients with limited treatment options

Day 120 opinion

Action: For adoption

Infectious Diseases

2.1.9. EMEA-002635-PIP01-19

Treatment of advanced or metastatic malignancies harbouring ALK, ROS1, or NTRK1-3 alterations

Day 120 opinion

Action: For adoption, Oral explanation to be held on 11 November 2020 at 17:00

Oncology

Note: Withdrawal request received on 02/11/2020

2.1.10. EMEA-002763-PIP01-20

Paediatric low grade glioma / Relapsed or refractory paediatric low grade glioma in adolescents and children 6 months of age and older

Day 120 opinion

Action: For adoption

Oncology

2.1.11. Tabelecleucel - Orphan - EMEA-002025-PIP04-19

Atara Biotherapeutics, Inc.; Treatment of Epstein-Barr virus associated post-transplant lymphoproliferative disorder / Treatment of allogeneic haematopoietic cell transplant patients with Epstein-Barr virus associated post-transplant lymphoproliferative disease who have received one prior therapy/ Treatment of solid organ transplant patients with Epstein-Barr virus associated post-transplant lymphoproliferative disease who have received one prior therapy

Day 120 opinion

Action: For adoption

Oncology

2.1.12. EMEA-002814-PIP01-20

Invasive disease caused by Neisseria meningitidis group A, B, C, W and Y from 2 months of age

Day 120 opinion

Action: For adoption

Vaccines

2.1.13. Bisoprolol / ramipril - EMEA-002860-PIP01-20

Essential hypertension

Day 60 opinion

Action: For adoption

Cardiovascular Diseases

2.1.14. Dapagliflozin - EMEA-000694-PIP06-20

Prevention of hospitalisation for heart failure and cardiovascular death in adults who have had a myocardial infarction

Day 60 opinion

Action: For adoption

Cardiovascular Diseases

2.1.15. Ziltivekimab - EMEA-002840-PIP01-20

Prevention of cardiovascular events in patients with atherosclerosis

Day 60 opinion

Action: For adoption

Cardiovascular Diseases

2.1.16. EMEA-002597-PIP03-20

Treatment of pemphigus

Day 60 opinion

Action: For adoption

Dermatology

2.1.17. Obinutuzumab - Orphan - EMEA-001207-PIP03-20

Roche Registration GmbH; Treatment of membranous glomerulonephritis also known as membranous nephropathy (MN) / product specific waiver request in all paediatric subsets

Day 60 opinion

Action: For adoption

Immunology-Rheumatology-Transplantation

2.1.18. EMEA-002866-PIP01-20

Treatment of idiopathic Parkinson's disease (PD)

Day 60 opinion

Action: For adoption

Neurology

2.1.19. Tauroursodeoxycholic acid / Sodium phenylbutyrate - Orphan - EMEA-002876-PIP01-20

Drug Development and Regulation SL; Treatment of amyotrophic lateral sclerosis

Day 60 opinion

Action: For adoption

Neurology

2.1.20. Delolimogene mupadenorepvec - Orphan - EMEA-002864-PIP01-20

Lokon Pharma AB; Colorectal cancer / Ovarian cancer / Pancreatic cancer / Malignant melanoma / Biliary cancer

Day 60 opinion

Action: For adoption

Oncology

2.1.21. Tipifarnib - EMEA-002871-PIP01-20

Treatment of HRAS mutant head and neck squamous cell carcinoma

Day 60 opinion

Action: For adoption

Oncology

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

Grifols Therapeutics LLC; Treatment of primary immunodeficiency

Day 60 opinion

Action: For adoption

Immunology-Rheumatology-Transplantation

2.3. Opinions on Modification of an Agreed Paediatric Investigation Plan

2.3.1. Captopril - EMEA-001544-PIP01-13-M02

Proveca Pharma Limited; Heart failure / Treatment of heart failure in children from birth to 18 years

Day 60 opinion

Action: For adoption

Cardiovascular Diseases

2.3.2. Landiolol (hydrochloride) - EMEA-001150-PIP02-13-M03

AOP Orphan Pharmaceuticals AG; Treatment of supraventricular arrythmias

Day 60 opinion

Action: For adoption

Cardiovascular Diseases

2.3.3. Fluciclovine (18F) - Orphan - EMEA-001644-PIP02-14-M02

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

Day 60 opinion

Action: For adoption

Diagnostic / Oncology

2.3.4. Cotadutide - EMEA-002287-PIP01-17-M01

AstraZeneca AB; Treatment of type 2 diabetes mellitus

Day 60 opinion

Action: For adoption

Endocrinology-Gynaecology-Fertility-Metabolism

2.3.5. EMEA-002642-PIP01-19-M02

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

Day 60 opinion

Action: For adoption

Endocrinology-Gynaecology-Fertility-Metabolism

Note: Withdrawal request received on 02/11/2020

2.3.6. Testosterone - EMEA-001529-PIP02-14-M03

Acerus Biopharma Inc.; Male hypogonadism

Day 60 opinion

Action: For adoption

Endocrinology-Gynaecology-Fertility-Metabolism

2.3.7. Naloxegol (as naloxegol oxalate) - EMEA-001146-PIP01-11-M06

Kyowa Kirin Pharmaceutical Development Limited; Treatment of opioid-induced constipation

Day 60 opinion

Action: For adoption

Gastroenterology-Hepatology

2.3.8. Avatrombopag maleate - EMEA-001136-PIP02-19-M01

Dova Pharmaceuticals Ireland Limited; Treatment of chemotherapy-induced thrombocytopenia / Treatment of chemotherapy-induced thrombocytopenia (CIT) in adult patients receiving myelosuppressive chemotherapy for solid tumours

Day 60 opinion

Action: For adoption

Haematology-Hemostaseology

2.3.9. Eltrombopag - EMEA-000170-PIP03-13-M04

Novartis Europharm Limited; Bone marrow depression and hypoplastic anaemia / Treatment of cytopenias in paediatric patients with severe aplastic anaemia who are not receiving hematopoietic stem cell transplant

Day 60 opinion

Action: For adoption

Haematology-Hemostaseology

2.3.10. Aztreonam - Orphan - EMEA-000827-PIP01-09-M05

Gilead Sciences International Ltd.; Cystic Fibrosis with pulmonary manifestations /

Treatment of initial Pseudomonas aeruginosa pulmonary infection/ colonisation in patients with cystic fibrosis/ Treatment of chronic Pseudomonas aeruginosa pulmonary infection/ colonisation in patients with cystic fibrosis

Day 60 opinion

Action: For adoption

Infectious Diseases

2.3.11. Cobicistat / atazanavir sulphate - EMEA-001465-PIP01-13-M03

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

Day 60 opinion

Action: For adoption

Infectious Diseases

2.3.12. Eculizumab - Orphan - EMEA-000876-PIP03-14-M05

Alexion Europe SAS; neuromyelitis optica spectrum disorders / Treatment of relapsing neuromyelitis optica spectrum disorders (NMOSD) in the paediatric population

Day 60 opinion

Action: For adoption

Neurology

2.3.13. Ocrelizumab - EMEA-000310-PIP03-10-M04

Roche Registration GmbH; Treatment of Multiple Sclerosis / Treatment of relapsing remitting multiple sclerosis

Day 60 opinion

Action: For adoption

Neurology

2.3.14. Avelumab - EMEA-001849-PIP02-15-M03

Merck Healthcare GmbH; Treatment of all conditions included in the category of malignant neoplasms (except central nervous system tumors, haematopoetic and lymphoid tissue neoplams)/ Treatment of malignant neoplasms of lymphoid tissue/ Treatment of malignant neoplasms of the central nervous system / Treatment of paediatric patients from 2 years to less than 18 years old with a relapsed or refractory solid tumour or with a solid tumour as part of the first line treatment

Day 60 opinion

Action: For adoption

Oncology

2.3.15. Brigatinib - EMEA-002296-PIP01-17-M02

Takeda Pharm A/S; Inflammatory myofibroblastic tumors (IMT) / Non-small cell lung cancer (NSCLC) / Anaplastic large cell lymphoma (ALCL) / Treatment of anaplastic lymphoma kinase (ALK) positive advanced non-small cell lung cancer (NSCLC) / Treatment of paediatric patients ≥1 years of age with ALK+ unresectable or recurrent IMT / Treatment in combination with standard chemotherapy in paediatric patients ≥1 years of age with newly diagnosed ALK+ ALCL at high risk for recurrence

Day 60 opinion

Action: For adoption

Oncology

2.3.16. Burosumab - Orphan - EMEA-001659-PIP01-15-M05

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

Day 60 opinion

Action: For adoption

Other

2.3.17. Ivacaftor / lumacaftor - EMEA-001582-PIP01-13-M10

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

Day 60 opinion

Action: For adoption

Other

2.3.18. Levofloxacin hemihydrate - EMEA-001211-PIP01-11-M02

Chiesi Farmaceutici S.p.A.; Cystic fibrosis

Day 60 opinion

Action: For adoption

Pneumology - Allergology

2.3.19. Calcifediol - EMEA-002093-PIP02-17-M01

Vifor Fresenius Medical Care Renal Pharma France; endocrine, nutritional and metabolic diseases, and immunity disorders

Day 60 opinion

Action: For adoption

Uro-nephrology

2.3.20. Cholera vaccine, recombinant, live, oral (strain CVD 103-HgR) - EMEA-001490-PIP01-13-M02

Emergent Netherlands B.V.; Cholera disease caused by Vibrio cholerae serogroup O1 / Prevention of disease caused by Vibrio cholerae serogroup O1

Day 60 opinion

Action: For adoption

Vaccines

2.4. Opinions on Re-examinations

No items

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. Bumetanide - EMEA-C1-001303-PIP01-12-M02

Les Laboratoires Servier; Treatment of autistic spectrum disorder

Day 30 discussion

Action: For information

Neurology

2.7.2. Maribavir - EMEA-C1-000353-PIP02-16-M01

Shire Pharmaceuticals Ireland Ltd; Treatment of cytomegalovirus (CMV) infection

Day 30 discussion

Action: For information

Infectious Diseases

2.7.3. Isavuconazonium (sulfate) - EMEA-C1-001301-PIP02-12-M03

Basilea Pharmaceutica International Ltd.; Treatment of mucormycosis

Day 30 discussion

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. EMEA-002757-PIP01-19

Treatment of ulcerative colitis

Day 90 discussion

Action: For discussion

Gastroenterology-Hepatology

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

Roche Registration GmbH; Systemic lupus erythemathosus

Day 90 discussion

Action: For discussion

Immunology-Rheumatology-Transplantation

3.1.3. EMEA-002740-PIP01-19

Treatment of human immunodeficiency virus (HIV-1) infection

Day 90 discussion

Action: For discussion

Infectious Diseases

3.1.4. Retinol (vitamin A) - Orphan - EMEA-002790-PIP01-20

Orphanix GmbH; Prevention of bronchopulmonary dysplasia (BPD)

Day 90 discussion

Action: For discussion

Neonatology - Paediatric Intensive Care

3.1.5. Arimoclomol citrate - Orphan - EMEA-001748-PIP03-19

Orphazyme A/S; Treatment of amyotrophic lateral sclerosis

Day 90 discussion

Action: For discussion

Neurology

3.1.6. Autologous tumor-infiltrating lymphocytes - EMEA-002776-PIP01-20

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

Day 90 discussion

Action: For discussion

Oncology

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

Amgen Europe BV; Treatment of acute lymphoblastic leukemia (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 one prior therapy or B-cell ALL treated with at least 2 prior therapies, with or without extramedullary disease

Day 90 discussion

Action: For discussion

Oncology / Haematology-Hemostaseology

3.1.8. KH176: (S)-6-hydroxy-2,5,7,8-tetramethyl-N-((R)-piperidin-3-yl)chroman-2-carboxamide hydrochloride - Orphan - EMEA-002113-PIP01-16

Khondrion BV; Treatment of mitochondrial respiratory chain / oxidative phosphorylation defects

Day 90 discussion

Action: For discussion

Other

3.1.9. Dexmedetomidine - EMEA-002758-PIP01-19

Treatment of acute agitation in bipolar disorder / Treatment of acute agitation in

schizophrenia

Day 90 discussion

Action: For discussion

Psychiatry

3.1.10. Esketamine - EMEA-002772-PIP01-20

Bipolar depression / Major depressive disorder / Treatment-resistant bipolar depression / Treatment-resistant depression in the course of major depressive disorder

Day 90 discussion

Action: For discussion

Psychiatry

3.1.11. Allopurinol / verinurad - EMEA-002754-PIP01-19

Chronic kidney disease / Treatment of chronic kidney disease in children and adolescents (6 to <18 years old) with hyperuricaemia and albuminuria

Day 90 discussion

Action: For discussion

Uro-nephrology

3.1.12. Sparsentan - Orphan - EMEA-001984-PIP02-20

Retrophin Europe Ltd; Treatment of focal segmental glomerular sclerosis (FSGS)

Day 90 discussion

Action: For discussion

Uro-nephrology

3.1.13. Sparsentan - EMEA-001984-PIP03-20

Treatment of IgA Nephropathy (IgAN)

Day 90 discussion

Action: For discussion

Uro-nephrology

3.1.14. EMEA-002870-PIP01-20

Induction of general anaesthesia in adults and children

Day 60 discussion

Action: For discussion

Anaesthesiology

3.1.15. Allogeneic skin-derived ABCB5-positive mesenchymal stem cells - Orphan - EMEA-002875-PIP01-20

RHEACELL GmbH & Co. KG; Treatment of epidermolysis bullosa / Treatment of recessive dystrophic epidermolysis bullosa and junctional epidermolysis bullosa

Day 60 discussion

Action: For discussion

Dermatology

3.1.16. Infigratinib - EMEA-002594-PIP02-20

Achondroplasia

Day 60 discussion

Action: For discussion

Endocrinology-Gynaecology-Fertility-Metabolism

3.1.17. Calmangfodipir - EMEA-002865-PIP01-20

paracetamol overdose in adolescents (more than 12 years) for increased risk (late presenters) to develop hepatoxicity

Day 60 discussion

Action: For discussion

Gastroenterology-Hepatology

3.1.18. Lanifibranor - EMEA-002872-PIP01-20

NASH / Treatment of non-alcoholic steatohepatitis (NASH)

Day 60 discussion

Action: For discussion

Gastroenterology-Hepatology

3.1.19. Odevixibat - Orphan - EMEA-002054-PIP03-20

Albireo AB; Alagille syndrome

Day 60 discussion

Action: For discussion

Gastroenterology-Hepatology

3.1.20. Vedolizumab - EMEA-000645-PIP04-20

Treatment of pouchitis

Day 60 discussion

Action: For discussion

Gastroenterology-Hepatology

3.1.21. EMEA-002863-PIP01-20

Paroxysmal nocturnal haemoglobinuria

Day 60 discussion

Action: For discussion

Haematology-Hemostaseology

3.1.22. Autologous CD4+ and CD8+ T cells transduced with a lentiviral vector containing an affinity-enhanced T-cell receptor targeting the MAGE-A4 antigen - Orphan - EMEA-002867-PIP01-20

Adaptimmune Ltd; Soft tissue sarcoma / Myxoid / Round cell liposarcoma / Synovial sarcoma

Day 60 discussion

Action: For discussion

Oncology

3.1.23. Magrolimab - Orphan - EMEA-002819-PIP01-20

Gilead Sciences International Ltd; Treatment of acute myeloid leukaemia / Treatment of myelodysplastic syndromes (including juvenile myelomonocytic leukaemia)

Day 60 discussion

Action: For discussion

Oncology

3.1.24. Pralsetinib - EMEA-002575-PIP02-20

Treatment of all conditions included in the category of malignant neoplasms (except haematopoietic and lymphoid tissue neoplasms) / Treatment of paediatric patients from 12 years to less than 18 years of age with RET-altered solid tumours who require systemic therapy and have no satisfactory alternative treatment options

Day 60 discussion

Action: For discussion

Oncology

3.1.25. Doconexent (Docosahexaenoic acid, DHA) - Orphan - EMEA-002808-PIP01-20

Natac Pharma S.L.; Retinitis pigmentosa

Day 60 discussion

Action: For discussion

Ophthalmology

3.1.26. (2S,4S)-2-(4-Carboxyphenyl)-4-ethoxy-1-[(5-methoxy-7-methyl-1H-indol-4-yl)methyl]piperidin-1-ium chloride—water (1/1) - Orphan - EMEA-002705-PIP03-20

Novartis Europharm Limited; Paroxysmal nocturnal haemoglobinuria

Day 60 discussion

Action: For discussion

Other / Haematology-Hemostaseology

3.1.27. Autologous selected renal cells - EMEA-002844-PIP01-20

Treatment of chronic kidney disease

Day 60 discussion

Action: For discussion

Uro-nephrology

3.1.28. EMEA-002873-PIP01-20

Active immunisation for the prevention of disease caused by chikungunya virus

Day 60 discussion

Action: For discussion

Vaccines

3.1.29. Rosuvastatin / acetylsalicylic acid - EMEA-002891-PIP01-20

Prevention of cardiovascular events

Day 30 discussion

Action: For discussion

Cardiovascular Diseases

3.1.30. Heparin - EMEA-002885-PIP01-20

Prevention and treatment of thromboembolic events

Day 30 discussion

Action: For discussion

Cardiovascular Diseases / Haematology-Hemostaseology

3.1.31. Human, recombinant, non-fucosylated IgG1k monoclonal antibody targeting OX-40 receptor on activated T cells - EMEA-002886-PIP01-20

Atopic dermatitis / Treatment of moderate to severe atopic dermatitis

Day 30 discussion

Action: For discussion

Dermatology

3.1.32. Semaglutide - EMEA-001441-PIP05-20

Treatment of non-alcoholic fatty liver disease (NAFLD)

Day 30 discussion

Action: For discussion

Gastroenterology-Hepatology

3.1.33. Fenebrutinib - EMEA-002349-PIP03-20

Treatment of multiple sclerosis / Treatment of relapsing multiple sclerosis in patients 10 years of age to less than 18 years of age

Day 30 discussion

Action: For discussion

Immunology-Rheumatology-Transplantation

3.1.34. EMEA-002861-PIP02-20

SARS-CoV-2 (COVID-19) infection prevention

Day 60 discussion

Action: For discussion

Article 7; PIP and Deferral and Waiver

Vaccines

3.1.35. Trimodulin - EMEA-002883-PIP01-20

Treatment of bacterial infections / Treatment of community-acquired sepsis/ Treatment of neonatal sepsis/ Treatment of severe community-acquired pneumonia/ Treatment of community-acquired septic shock/ Treatment of community-acquired severe sepsis

Day 30 discussion

Action: For discussion

Neonatology - Paediatric Intensive Care / Infectious Diseases / Pneumology - Allergology

3.1.36. Zidebactam / cefepime - EMEA-002892-PIP01-20

Treatment of complicated Urinary Tract Infections (cUTI)

Day 30 discussion

Action: For discussion

Neonatology - Paediatric Intensive Care / Infectious Diseases / Uro-nephrology

3.1.37. Edasalonexent [N-(2-((4Z,7Z,10Z,13Z,16Z,19Z)-docosa-4,7,10,13,16,19-hexaenamido)ethyl)-2-hydroxybenzamide] - Orphan - EMEA-001960-PIP03-20

Catabasis Pharmaceuticals Inc.; Duchenne muscular dystrophy / Treatment of Duchenne muscular dystrophy

Day 30 discussion

Action: For discussion

Neurology

3.1.38. Ravulizumab - EMEA-001943-PIP03-20

Acetylcholine receptor-antibody positive generalized myasthenia gravis / Treatment of acetylcholine receptor-antibody positive generalized myasthenia gravis

Day 30 discussion

Action: For discussion

Neurology

3.1.39. Reldesemtiv - Orphan - EMEA-002868-PIP01-20

Cytokinetics, Inc.; Amyotrophic lateral sclerosis (ALS)

Day 30 discussion

Action: For discussion

Neurology

3.1.40. EMEA-002884-PIP01-20

Progressive supranuclear palsy

Day 30 discussion

Action: For discussion

Neurology

3.1.41. EMEA-002745-PIP02-20

Treatment of lymphoplasmacytic lymphoma

Day 30 discussion

Action: For discussion

Oncology

3.1.42. Allogeneic anti-CD19 CAR T-cells produced using CRISPR/Cas9 to disrupt the T-cell receptor alpha constant (TRAC) and β2-microglobulin (B2M) genomic loci and a recombinant adeno-associated viral vector to deliver donor template for insertion of the anti-CD19 CAR expression cassette into the TRAC locus - EMEA-002881-PIP01-20

B cell lymphoblastic leukemia/lymphoma / Mature B cell neoplasms / Treatment of relapsed/refractory B cell ALL / Treatment of relapsed/refractory B cell NHL

Day 30 discussion

Action: For discussion

Oncology

3.1.43. 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-PIP03-20

Kite Pharma EU B.V.; Treatment of mature B-cell neoplasms / Treatment of paediatric and adolescent subjects with relapsed or refractory B-cell non-Hodgkin lymphoma (NHL)

Day 30 discussion

Action: For discussion

Oncology

3.1.44. Catumaxomab - EMEA-002879-PIP01-20

Treatment of malignant ascites

Day 30 discussion

Action: For discussion

Oncology

3.1.45. Lorlatinib - EMEA-002669-PIP02-20

Treatment of lung malignant neoplasms

Day 30 discussion

Action: For discussion

Oncology

3.1.46. Ublituximab - Orphan - EMEA-002889-PIP01-20

CambPharma Solutions (CY) Ltd; Treatment of chronic lymphocytic leukemia (CLL)

Day 30 discussion

Action: For discussion

Oncology

3.1.47. Umbralisib - EMEA-002890-PIP01-20

Treatment chronic lymphocytic leukemia (CLL)

Day 30 discussion

Action: For discussion

Oncology

3.1.48. Ribitol - EMEA-002887-PIP01-20

Treatment of Limb-Girdle muscular dystrophy / Treatment of paediatric patients aged 5 years and older with a genetically confirmed diagnosis of Limb-Girdle muscular dystrophy type 2i (LGMD2I) / LGMD R9-fukutin related

Day 30 discussion

Action: For discussion

Other

3.1.49. Paracetamol / nefopam hydrochloride - EMEA-002877-PIP01-20

Treatment of acute pain / Symptomatic short-term treatment of moderate to severe somatic acute pain

Day 30 discussion

Action: For discussion

Pain

3.1.50. Eliapixant - EMEA-002882-PIP01-20

Treatment of refractory and/or unexplained chronic cough (RUCC) Day 30 discussion

Action: For discussion

Pneumology - Allergology

3.1.51. Alpha1-Proteinase Inhibitor (Human) - EMEA-002888-PIP01-20

Treatment of emphysema secondary to alpha 1-proteinase inhibitor deficiency

Day 30 discussion

Action: For discussion

Pneumology - Allergology / Haematology-Hemostaseology

3.1.52. Acetylcysteine / Ibuprofen - EMEA-002561-PIP02-20

Upper respiratory tract signs and symptoms

Day 30 discussion

Action: For discussion

Pneumology - Allergology / Oto-rhino-laryngology

3.1.53. mRNA that encodes for the pre-fusion stabilized Spike glycoprotein of 2019-novel Coronavirus - EMEA-002893-PIP01-20

Prevention of COVID-19 / Active immunisation against SARS-CoV-2

Day 30 discussion

Action: For discussion

Vaccines

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. Riociguat - EMEA-C-000718-PIP01-09-M06

Bayer AG; Treatment of pulmonary hypertension

Day 30 discussion

Action: For discussion

Cardiovascular Diseases

3.2.2. Idursulfase - EMEA-C-000294-PIP02-12-M01

Shire Human Genetic Therapies AB; Treatment of mucopolysaccharidosis II (Hunter syndrome)

Day 30 discussion

Action: For discussion

Endocrinology-Gynaecology-Fertility-Metabolism

3.2.3. Bictegravir / Tenofovir alafenamide / emtricitabine - EMEA-C2-001766-PIP01-15-M02

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

Day 30 discussion

Action: For discussion

Infectious Diseases

3.2.4. Elbasvir / grazoprevir - EMEA-C-001604-PIP01-13-M03

Merck Sharp & Dohme B.V.; Treatment of chronic hepatitis C

Day 30 discussion

Action: For discussion

Infectious Diseases

3.2.5. Burosumab - Recombinant Human IgG1 monoclonal antibody targeting fibroblast growth factor 23 (FGF23) - EMEA-C4-001659-PIP01-15-M04

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

Day 30 discussion

Action: For discussion

Other

3.3. Discussions on Modification of an Agreed Paediatric Investigation Plan

3.3.1. Remimazolam (as besylate) - EMEA-001880-PIP02-19-M01

PAION Deutschland GmbH; Sedation / General anesthesia / Sedation of mechanically ventilated patients / Procedural sedation

Day 30 discussion

Action: For discussion

Anaesthesiology

3.3.2. Denosumab - EMEA-000145-PIP02-12-M03

Amgen Europe B.V.; Treatment of Osteoporosis / Treatment of osteogenesis imperfecta / Treatment of glucocortcoid induced osteoporosis

Day 30 discussion

Action: For discussion

Endocrinology-Gynaecology-Fertility-Metabolism

3.3.3. Darvadstrocel - Orphan - EMEA-001561-PIP01-13-M02

Takeda Pharma A/S; Perianal fistula

Day 30 discussion

Action: For discussion

Gastroenterology-Hepatology

3.3.4. Obeticholic acid - Orphan - EMEA-001304-PIP02-13-M05

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

Day 30 discussion

Action: For discussion

Gastroenterology-Hepatology

3.3.5. Tofacitinib - EMEA-000576-PIP03-12-M05

Pfizer Europe MA EEIG; Treatment of ulcerative colitis

Day 30 discussion

Action: For discussion

Gastroenterology-Hepatology

3.3.6. Betibeglogene autotemcel - Orphan - EMEA-001665-PIP01-14-M04

bluebird bio (Netherlands) B.V.; Treatment of β-thalassaemia / Treatment of beta-thalassaemia major and severe intermedia

Day 30 discussion

Action: For discussion

Haematology-Hemostaseology

3.3.7. Luspatercept - Orphan - EMEA-001521-PIP01-13-M05

Celgene Europe B.V.; Treatment of beta-thalassaemia / Treatment of anaemia in patients with beta-thalassemia intermedia and major

Day 30 discussion

Action: For discussion

Haematology-Hemostaseology

3.3.8. Risankizumab - EMEA-001776-PIP02-17-M01

AbbVie Ltd; Chronic idiopathic arthritis

Day 30 discussion

Action: For discussion

Immunology-Rheumatology-Transplantation

3.3.9. Tofacitinib citrate - EMEA-000576-PIP01-09-M13

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

Day 30 discussion

Action: For discussion

Immunology-Rheumatology-Transplantation

3.3.10. 3-({5-chloro-1-[3-(methylsulfonyl)propyl]-1Hindol-2-yl}methyl)-1-(2,2,2-trifluoroethyl)-1,3-dihydro-2H-imidazo[4,5-C]pyridin-2-one) (JNJ-53718678) - EMEA-001838-PIP01-15-M03

Janssen-Cilag International NV; Treatment of respiratory tract disease caused by human respiratory syncytial virus (RSV)

Day 30 discussion

Action: For discussion

Infectious Diseases

3.3.11. Aztreonam / Avibactam - EMEA-002283-PIP01-17-M01

Pfizer Europe MA EEIG; Treatment of infections caused by aerobic gram-negative bacteria / Treatment of infections caused by aerobic gram-negative bacteria in patients with limited therapeutic options

Day 30 discussion

Action: For discussion

Infectious Diseases

3.3.12. Baloxavir marboxil - EMEA-002440-PIP01-18-M01

Roche Registration GmbH; Prevention of Influenza / Treatment of Influenza / Treatment of influenza type A/B in otherwise healthy and high risk patients / Prevention (post-exposure prophylaxis) of influenza type A/B. Reduction of transmission of influenza type A/B

Day 30 discussion

Action: For discussion

Infectious Diseases

3.3.13. Ceftazidime / avibactam - EMEA-001313-PIP01-12-M10

Pfizer Europe MA EEIG; Treatment of bacterial infections / Treatment of complicated urinary tract infections / Treatment of complicated intraabdominal infections / Treatment of pneumonia / Treatment of infections due to aerobic Gram-negative organisms

Day 30 discussion

Action: For discussion

Infectious Diseases

3.3.14. Cobicistat - EMEA-000969-PIP01-10-M05

Gilead Sciences International Ltd.; Treatment of human immunodeficiency virus type-1 (HIV-1) infection / Treatment of human immunodeficiency virus type-1 (HIV-1) infection - pharmacoenhancer for use in combination with antiretroviral agents

Day 30 discussion

Action: For discussion

Infectious Diseases

3.3.15. Cobicistat / darunavir - EMEA-001280-PIP01-12-M03

Janssen-Cilag International NV; Treatment of HIV-1 infection / Treatment of HIV-1 infection in paediatric patients from 3 to less than 18 years

Day 30 discussion

Action: For discussion

Infectious Diseases

3.3.16. Tenofovir alafenamide / Emtricitabine - EMEA-001577-PIP02-14-M04

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

Day 30 discussion

Action: For discussion

Infectious Diseases

3.3.17. Tenofovir alafenamide / Emtricitabine / Bictegravir - EMEA-001766-PIP01-15-M03

Gilead Sciences International Ltd.; Treatment of Human immunodeficiency virus [HIV] disease resulting in other conditions / treatment of adults and paediatrics aged less than 2 years weighing more than 4 kg infected with human immunodeficiency virus-1 (HIV-1) without any known mutations associated with resistance to the individual components

Day 30 discussion

Action: For discussion

Infectious Diseases

3.3.18. Cannabidiol - Orphan - EMEA-001964-PIP01-16-M03

GW Pharma (International) B.V.; Lennox Gastaut Syndrome / Tuberous sclerosis complex / Infantile spasms / Dravet Syndrome / Treatment of seizures

Day 30 discussion

Action: For discussion

Neurology

3.3.19. Lasmiditan - EMEA-002166-PIP01-17-M05

Eli Lilly and Company Limited; Migraine with and without aura

Day 30 discussion

Action: For discussion

Neurology

3.3.20. Ofatumumab - EMEA-002397-PIP01-18-M01

Novartis Europharm Limited; Treatment of multiple sclerosis / Treatment of relapsing remitting multiple sclerosis

Day 30 discussion

Action: For discussion

Neurology

3.3.21. Pitolisant - Orphan - EMEA-001176-PIP01-11-M06

BIOPROJET PHARMA; Narcolepsy / Narcolepsy with or without cataplexy

Day 30 discussion

Action: For discussion

Neurology

3.3.22. Siponimod hemifumarate - EMEA-000716-PIP01-09-M03

Novartis Europharm Ltd; Treatment of multiple sclerosis / Treatment of children/adolescent patients (10-18 years old) with relapsing forms of multiple sclerosis

Day 30 discussion

Action: For discussion

Neurology

3.3.23. Brentuximab vedotin - Orphan - EMEA-000980-PIP01-10-M07

Takeda Pharma A/S; Treatment of Hodgkin lymphoma / Treatment of paediatric patients with newly diagnosed relapse or refractory Hodgkin lymphoma (from 5 years of age)

Day 30 discussion

Action: For discussion

Oncology

3.3.24. Crizotinib - EMEA-001493-PIP03-18-M01

Pfizer Europe MA EEIG; Inflammatory myofibroblastic tumour (IMT) / Anaplastic large cell lymphoma (ALCL) / Treatment of paediatric patients that are able to swallow capsules (age range: 6 years to less than 18 years of age) with relapsed/refractory systemic ALK-positive ALCL / Treatment of paediatric patients that are able to swallow capsules (age range: 6 years to less than 18 years of age) with unresectable or relapsed/refractory ALK-positive IMT / Treatment of paediatric patients with relapsed/refractory systemic ALK-positive ALCL / Treatment of paediatric patients with unresectable or relapsed/refractory ALK-positive IMT

Day 30 discussion

Action: For discussion

Oncology

3.3.25. Eribulin - EMEA-001261-PIP01-11-M06

Eisai GmbH; Soft tissue sarcoma

Day 30 discussion

Action: For discussion

Oncology

3.3.26. Fosdenopterin - Orphan - EMEA-001491-PIP01-13-M01

Origin Biosciences, Inc.; Treatment of molybdenum cofactor deficiency type A

Day 30 discussion

Action: For discussion

Other

3.3.27. Human thrombin (component 2) / Human fibrinogen (component 1) - EMEA-001598-PIP01-13-M03

Instituto Grifols, S.A.; Treatment of haemorrhage resulting from a surgical procedure / Supportive treatment in surgery where standard surgical techniques are insufficient for improvement of haemostasis, and as a suture support in vascular surgery

Day 30 discussion

Action: For discussion

Other

3.3.28. Human Thrombin / Human Fibrinogen - EMEA-001149-PIP01-11-M06

Omrix Biopharmaceuticals N.V.; Treatment of haemorrhage resulting from a surgical procedure / Treatment of cerebrospinal fluid leakage resulting from a surgical procedure / for supportive treatment in surgery where standard surgical techniques are insufficient, for improvement of haemostasis / for suture line sealing in dura mater closure / for supportive treatment in surgery where standard surgical techniques are insufficient / for improvement of haemostasis

Day 30 discussion

Action: For discussion

Other

3.3.29. In vitro expanded autologous human articular chondrocytes - EMEA-001823-PIP01-15-M02

TETEC Tissue Engineering Technologies AG; Treatment of cartilage disorders

Day 30 discussion

Action: For discussion

Other

3.3.30. In vitro expanded autologous human articular chondrocytes - EMEA-002217-PIP01-17-M01

TETEC Tissue Engineering Technologies AG; Treatment of cartilage disorders

Day 30 discussion

Action: For discussion

Other

3.3.31. Vortioxetine - EMEA-000455-PIP02-10-M07

H. Lundbeck A/S; Major depressive disorder

Day 30 discussion

Action: For discussion

Psychiatry

3.3.32. Daprodustat - EMEA-001452-PIP01-13-M03

GlaxoSmithKline Trading Services Limited; Treatment of anaemia associated with chronic

kidney disease

Day 30 discussion

Action: For discussion

Uro-nephrology / Haematology-Hemostaseology

3.3.33. 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-M02

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 submission of applications with start of procedure 1st December 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.

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

None

7. Discussion on the inclusion of an indication within a condition in an agreed PIP/waiver

7.1. Discussion on the possibility to include an indication within a condition in an agreed PIP/waiver

None

8. Annual reports on deferrals

Note: The annual reports on deferrals to be noted by the members of the PDCO are flagged in the Annex B.

9. Organisational, regulatory and methodological matters

9.1. Mandate and organisation of the PDCO

9.1.1. Strategic Review & Learning Meeting – PDCO, 22 October, Germany

Action: For information

9.2. Coordination with EMA Scientific Committees or CMDh-v

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

Action: For information

9.3. 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

None

9.5. Cooperation with International Regulators

None

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

None

9.7. PDCO work plan

None

9.8. Planning and reporting

None

10. Any other business

10.1.1. COVID -19 update

Action: For information

10.1.2. EMA IT tool on marketing status information

Action: For information

10.1.3. PDCO Meeting dates 2022-2024

Action: For information

10.1.4. Template for PDCO Opinion - Proposed revision of the extrapolation related section

Action: For information

10.1.5. PDCO Member at the HMPC

Action: For information

10.1.6. PDCO Eudramail mailbox - update

Action: For information

11. Breakout sessions

11.1.1. Neonatology

Action: For discussion on Thursday, 13:00-14:00

11.1.2. Duchenne Muscular Dystrophy

Action: For discussion on Tuesday, 13:00-14:00

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/