



EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

13 October 2020
EMA/PDCO/490611/2020
Human Medicines Division

Paediatric Committee (PDCO)

Agenda for the meeting on 13-16 October 2020

Chair: Koenraad Norga – Vice-Chair: Sabine Scherer

13 October 2020, 14:00- 19:00, Virtual meeting

14 October 2020, 08:30- 19:00, Virtual meeting

15 October 2020, 08:30- 19:00, Virtual meeting

16 October 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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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 13-16 October 2020. See 13-16 October 2020 PDCO minutes (to be published post 10-13 November 2020 PDCO meeting).

1.2. Adoption of agenda

PDCO agenda for 13-16 October 2020

1.3. Adoption of the minutes

PDCO minutes for 1-4 September 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. 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 120 opinion

Action: For adoption

Endocrinology-Gynaecology-Fertility-Metabolism

2.1.2. Venglustat - Orphan - EMEA-001716-PIP04-19

Genzyme Europe B.V.; GM2 gangliosidosis, Other gangliosidosis, GM1, GM3, 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 120 opinion

Action: For adoption

Endocrinology-Gynaecology-Fertility-Metabolism / Neurology

2.1.3. [Guselkumab - EMEA-001523-PIP04-19](#)

Ulcerative Colitis / Treatment of Ulcerative Colitis

Day 120 opinion

Action: For adoption

Gastroenterology-Hepatology

2.1.4. [Bispecific antibody binding to clotting factor IX and X - EMEA-002762-PIP02-20](#)

Treatment of haemophilia A / Routine prophylaxis to prevent or reduce frequency of bleeding episodes in patients with haemophilia A (congenital factor VIII deficiency) with or without factor VIII inhibitors.

Day 120 opinion

Action: For adoption

Haematology-Hemostaseology

2.1.5. [Garadacimab - EMEA-002726-PIP01-19](#)

Hereditary angioedema attacks (HAE)

Day 120 opinion

Action: For adoption

Haematology-Hemostaseology

2.1.6. [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 120 opinion

Action: For adoption

Immunology-Rheumatology-Transplantation

2.1.7. [Alpha1-proteinase inhibitor \(human\) - EMEA-001312-PIP03-19](#)

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

Day 120 opinion

Action: For adoption

2.1.8. Bimekizumab - EMEA-002189-PIP03-19

Treatment of chronic idiopathic arthritis (including rheumatoid arthritis, spondyloarthritis, psoriatic arthritis and juvenile idiopathic arthritis) / Treatment of JIA (enthesitis-related arthritis [ERA] and juvenile psoriatic arthritis [JPsA]) in patients from ≥ 2 years to < 18 years of age

Day 120 opinion

Action: For adoption

Immunology-Rheumatology-Transplantation

2.1.9. Cotadutide - EMEA-002712-PIP01-19

Treatment of non-cirrhotic non-alcoholic steatohepatitis (NASH) with fibrosis / Treatment of non-cirrhotic non-alcoholic steatohepatitis (NASH) with fibrosis in children and adolescents

Day 120 opinion

Action: For adoption

Immunology-Rheumatology-Transplantation

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

Treatment of systemic lupus erythematosus (SLE) / Treatment of children and adolescents ≥ 5 years to < 18 years of age with active SLE despite standard therapy

Day 120 opinion

Action: For adoption

Immunology-Rheumatology-Transplantation

2.1.11. Taniborbactam / cefepime - EMEA-002576-PIP01-19

Treatment of gram-negative bacterial infections / Treatment of cUTI including acute pyelonephritis

Day 120 opinion

Action: For adoption

Infectious Diseases

2.1.12. Vonoprazan - EMEA-002703-PIP01-19

Treatment of reflux oesophagitis / Treatment of Helicobacter pylori infection / Eradication of H. pylori concurrently given with appropriate antibiotic therapy / Treatment of erosive reflux oesophagitis / Maintenance of healed erosive reflux oesophagitis

Day 120 opinion

Action: For adoption

Infectious Diseases / Gastroenterology-Hepatology

2.1.13. Adeno-associated viral vector serotype 9 containing the human mini-dystrophin gene (PF-06939926) - Orphan - EMEA-002741-PIP01-20

Pfizer Europe MA EEIG; Treatment of Duchenne Muscular Dystrophy

Day 120 opinion

Action: For adoption

Neurology

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

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

Day 120 opinion

Action: For adoption

Other

2.1.15. Adrenaline (epinephrine) - EMEA-002749-PIP01-19

Treatment of allergic reactions / The emergency treatment of allergic reactions, including anaphylaxis

Day 120 opinion

Action: For adoption, Oral explanation to be held on 14 October 2020 at 17:00

Pneumology - Allergology

2.1.16. BI 425809 - EMEA-002653-PIP01-19

Treatment of Schizophrenia / Treatment of cognitive impairment associated with schizophrenia in patients 13 to <18 years of age

Day 120 opinion

Action: For adoption

Psychiatry

2.1.17. Ezetimibe / Atorvastatin - EMEA-002852-PIP01-20

Prevention of cardiovascular events / Treatment of hypercholesterolemia / The combination of Atorvastatin and Ezetimibe is indicated for the treatment of hypercholesterolemia as

substitution therapy in adult patients adequately controlled with the individual substances given concurrently at the same dose level as in the fixed dose combination, but as separate products / Atorvastatin/Ezetimibe Sandoz is indicated as substitution therapy to reduce the risk of cardiovascular events in patients with coronary heart disease (CHD) and a history of acute coronary syndrome (ACS), for adults receiving atorvastatin and ezetimibe concurrently at the same dose level.

Day 60 opinion

Action: For adoption

Cardiovascular Diseases

2.1.18. [Perindopril arginine / Indapamide / Amlodipine besilate - EMEA-002849-PIP01-20](#)

Hypertension

Day 60 opinion

Action: For adoption

Cardiovascular Diseases

2.1.19. [Chimeric fibrin-reactive IgG1k monoclonal antibody 11-1F4 - Orphan - EMEA-002791-PIP01-20](#)

Real Regulatory Limited; AL Amyloidosis / Treatment of AL Amyloidosis

Day 60 opinion

Action: For adoption

Endocrinology-Gynaecology-Fertility-Metabolism

2.1.20. [Fasudil hydrochloride - EMEA-002841-PIP01-20](#)

Non-traumatic subarachnoid haemorrhage

Day 60 opinion

Action: For adoption

Neurology

2.1.21. [Allogeneic BCMA-directed chimeric antigen receptor T Cell - EMEA-002834-PIP01-20](#)

Multiple myeloma

Day 60 opinion

Action: For adoption

Oncology

2.1.22. Lurbinectedin - Orphan - EMEA-002846-PIP01-20

Pharma Mar, S.A.; Treatment of small cell lung cancer

Day 60 opinion

Action: For adoption

Oncology

2.1.23. Serplulimab - EMEA-002859-PIP01-20

Lung cancer (SCLC and NSCLC)

Day 60 opinion

Action: For adoption

Oncology

2.1.24. EMEA-002843-PIP01-20

Treatment of ovarian cancer

Day 60 opinion

Action: For adoption

Oncology / Haematology-Hemostaseology

2.1.25. EMEA-002843-PIP02-20

Treatment of breast cancer

Day 60 opinion

Action: For adoption

Oncology / Haematology-Hemostaseology

2.1.26. (1R,2R,3S,4S,5R,6S)-cyclohexane-1,2,3,4,5,6-hexayl-hexakis (dihydrogen phosphate) - Orphan - EMEA-002854-PIP01-20

Sanifit Therapeutics S.A.; Treatment of calciphylaxis

Day 60 opinion

Action: For adoption

Other

2.1.27. Dapagliflozin - EMEA-000694-PIP05-20

COVID-19, virus identified, COVID-19, virus not identified

Day 60 opinion

Action: For adoption

Other

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. Sofosbuvir / Voxilaprevir / Velpatasvir - EMEA-C-001822-PIP01-15-M01

Gilead Sciences Ireland UC; Treatment of chronic hepatitis C

Day 60 opinion

Action: For adoption

Infectious Diseases

2.2.2. Regorafenib - EMEA-C2-001178-PIP01-11-M05

Bayer AG; Treatment of all conditions contained in the category of malignant neoplasms (except haematopoietic and lymphoid tissue)

Day 60 letter

Action: For adoption

Oncology

2.2.3. Pneumococcal polysaccharide serotype 5, conjugated to CRM197 carrier protein and adsorbed on aluminium phosphate / Pneumococcal polysaccharide serotype 19A, conjugated to CRM197 carrier protein and adsorbed on aluminium phosphate / Pneumococcal polysaccharide serotype 33F, conjugated to CRM197 carrier protein and adsorbed on aluminium phosphate / Pneumococcal polysaccharide serotype 9V, conjugated to CRM197 carrier protein and adsorbed on aluminium phosphate / Pneumococcal polysaccharide serotype 8, conjugated to CRM197 carrier protein and adsorbed on aluminium phosphate / Pneumococcal polysaccharide serotype 19F, conjugated to CRM197 carrier protein and adsorbed on aluminium phosphate / Pneumococcal polysaccharide serotype 23F, conjugated to CRM197 carrier protein and adsorbed on aluminium phosphate / Pneumococcal polysaccharide serotype 10A, conjugated to CRM197 carrier protein and adsorbed on aluminium phosphate / Pneumococcal polysaccharide serotype 7F, conjugated to CRM197 carrier protein and adsorbed on aluminium phosphate / Pneumococcal polysaccharide serotype 15B, conjugated to CRM197 carrier protein and adsorbed on aluminium phosphate / Pneumococcal polysaccharide serotype 18C, conjugated to CRM197 carrier protein and adsorbed on aluminium phosphate / Pneumococcal polysaccharide serotype 6B, conjugated to CRM197 carrier protein and adsorbed on aluminium phosphate / Pneumococcal polysaccharide serotype 6A, conjugated to CRM197 carrier protein and adsorbed on aluminium phosphate / Pneumococcal polysaccharide serotype 3, conjugated to CRM197 carrier protein and adsorbed on aluminium phosphate / Pneumococcal polysaccharide serotype 4, conjugated to CRM197 carrier protein and adsorbed on aluminium phosphate / Pneumococcal polysaccharide serotype 11A, conjugated to CRM197 carrier protein and adsorbed on aluminium phosphate / Pneumococcal polysaccharide serotype 1, conjugated to CRM197 carrier protein and

adsorbed on aluminium phosphate / Pneumococcal polysaccharide serotype 12F, conjugated to CRM197 carrier protein and adsorbed on aluminium phosphate / Pneumococcal polysaccharide serotype 22F, conjugated to CRM197 carrier protein and adsorbed on aluminium phosphate / Pneumococcal polysaccharide serotype 14, conjugated to CRM197 carrier protein and adsorbed on aluminium phosphate -
EMA-C1-002330-PIP01-18

Pfizer Europe MA EEIG; Disease caused by *Streptococcus pneumoniae*

Day 60 letter

Action: For adoption

Vaccines

2.3. Opinions on Modification of an Agreed Paediatric Investigation Plan

2.3.1. Bilastine - EMA-000347-PIP02-16-M02

Faes Farma S.A.; Treatment of allergic conjunctivitis

Day 60 opinion

Action: For adoption

2.3.2. Tralokinumab - EMA-001900-PIP02-17-M04

LEO Pharma A/S; Treatment of Atopic Dermatitis

Day 60 opinion

Action: For adoption

Dermatology

2.3.3. Canagliflozin - EMA-001030-PIP01-10-M08

Janssen-Cilag International NV; Type 2 Diabetes Mellitus / Treatment of Type 2 Diabetes Mellitus

Day 60 opinion

Action: For adoption

Endocrinology-Gynaecology-Fertility-Metabolism

2.3.4. Drospirenone / Estetrol monohydrate - EMA-001332-PIP01-12-M04

Estetra SPRL; Prevention of pregnancy

Day 60 opinion

Action: For adoption

Endocrinology-Gynaecology-Fertility-Metabolism

2.3.5. Lixisenatide - EMEA-000916-PIP01-10-M07

sanofi-aventis R&D; Type 2 diabetes mellitus / Treatment of type 2 diabetes mellitus

Day 60 opinion

Action: For adoption

Endocrinology-Gynaecology-Fertility-Metabolism

2.3.6. Olipudase alfa - Orphan - EMEA-001600-PIP01-13-M02

Genzyme Europe B.V.; Endocrine, nutritional and metabolic diseases / Metabolic disorders / Disorders of sphingolipid metabolism and other lipid storage disorders / Other sphingolipidosis / Niemann-Pick Disease / Treatment of Niemann-Pick Disease

Day 60 opinion

Action: For adoption

Endocrinology-Gynaecology-Fertility-Metabolism

2.3.7. Recombinant parathyroid hormone: rhPTH (1-84) - Orphan - EMEA-001526-PIP01-13-M04

Shire Pharmaceuticals Ireland Limited; Hypoparathyroidism / Treatment of hypoparathyroidism

Day 60 opinion

Action: For adoption

Endocrinology-Gynaecology-Fertility-Metabolism

2.3.8. Potassium Chloride / Sodium Chloride / Ascorbic Acid / Sodium Sulfate / Sodium Ascorbate / Polyethylene Glycol 3350 - EMEA-001705-PIP02-15-M03

Norgine Limited; Bowel cleansing prior to any procedure requiring a clean bowel / Bowel cleansing prior to clinical procedures

Day 60 opinion

Action: For adoption

Gastroenterology-Hepatology

2.3.9. Teduglutide - Orphan - EMEA-000482-PIP01-08-M06

Shire Pharmaceuticals Ireland Limited; Syndrome Short Bowel / Treatment of Short Bowel Syndrome

Day 60 opinion

Action: For adoption

Gastroenterology-Hepatology

2.3.10. Vonicog alfa - EMEA-001164-PIP01-11-M04

Baxalta Innovations GmbH; Von Willebrand disease / Prevention and treatment of bleeding episodes and for surgical and invasive procedures in paediatric patients (less than 18 years of age) with von Willebrand disease

Day 60 opinion

Action: For adoption

Haematology-Hemostaseology

2.3.11. Apremilast - EMEA-000715-PIP03-11-M06

Amgen Europe B.V.; Treatment of psoriasis / Treatment of moderate to severe psoriasis

Day 60 opinion

Action: For adoption

Immunology-Rheumatology-Transplantation

2.3.12. Ustekinumab - EMEA-000311-PIP03-11-M06

Janssen-Cilag International NV; Treatment of chronic idiopathic arthritis (including rheumatoid arthritis / spondyloarthritis / PsA and juvenile idiopathic arthritis [JIA]) / Treatment of juvenile idiopathic arthritis (juvenile psoriatic arthritis [jPsA])

Day 60 opinion

Action: For adoption

Immunology-Rheumatology-Transplantation

2.3.13. Tazobactam / ceftolozane - EMEA-001142-PIP01-11-M04

Merck Sharp & Dohme (Europe), Inc.; Treatment of urinary tract infections, Treatment of intra-abdominal infections / Treatment of complicated urinary tract infections (cUTI) / Treatment of complicated intra-abdominal infections (cIAI)

Day 60 opinion

Action: For adoption

Infectious Diseases

2.3.14. Tedizolid - EMEA-001379-PIP01-12-M05

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

Day 60 opinion

Action: For adoption

Infectious Diseases

2.3.15. [Tenofovir Alafenamide / Emtricitabine / Elvitegravir / Cobicistat - EMEA-001460-PIP01-13-M05](#)

Gilead Sciences International Ltd.; Treatment of human immunodeficiency virus (HIV-1) infection / Genvoya is indicated for the treatment of HIV-1 infection in paediatric patients from 6 years to less than 18 years.

Day 60 opinion

Action: For adoption

Infectious Diseases

2.3.16. [Tenofovir disoproxil \(as fumarate\) - EMEA-000533-PIP01-08-M09](#)

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 60 opinion

Action: For adoption

Infectious Diseases

2.3.17. [Fenfluramine hydrochloride - Orphan - EMEA-001990-PIP01-16-M03](#)

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

Day 60 opinion

Action: For adoption

Neurology

2.3.18. [Humanised anti-IL-6 receptor \(IL-6R\) monoclonal antibody \(satralizumab\) - Orphan - EMEA-001625-PIP01-14-M06](#)

Roche Registration GmbH; Treatment of neuromyelitis optica

Day 60 opinion

Action: For adoption

Neurology

2.3.19. [Nivolumab - EMEA-001407-PIP01-12-M03](#)

Bristol-Myers Squibb Pharma EEIG; Treatment of all conditions included in the category of malignant neoplasms (except nervous system, haematopoietic and lymphoid tissue) / Treatment of patients with unresectable or metastatic melanoma in the age group from 12 to less than 18 years old / Treatment of a paediatric malignant solid tumour in paediatric patients from 6 months to less than 18 years old

Day 60 opinion

Action: For adoption

Oncology

2.3.20. Nivolumab - EMEA-001407-PIP02-15-M04

Bristol-Myers Squibb Pharma EEIG; Treatment of malignant neoplasms of lymphoid tissue / Treatment of malignant neoplasms of the central nervous system / Treatment of paediatric patients with a relapsed or refractory non-Hodgkin lymphoma in the age group from 6 months to less than 18 years old / Treatment of paediatric patients from 6 months to less than 18 years of age with a recurrent or progressive high-grade glioma / Treatment of paediatric patients with a relapsed or refractory Hodgkin lymphoma in the age group from 5 years to less than 18 years

Day 60 opinion

Action: For adoption

Oncology

2.3.21. Veliparib - EMEA-000499-PIP02-10-M01

AbbVie Ltd; Treatment of high grade glioma

Day 60 opinion

Action: For adoption

Oncology

2.3.22. Concentrate of proteolytic enzyme enriched in bromelain - Orphan - EMEA-000142-PIP02-09-M10

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

Day 60 opinion

Action: For adoption

Other

2.3.23. Palovarotene - Orphan - EMEA-001662-PIP01-14-M04

Ipsen Pharma; Treatment of fibrodysplasia ossificans progressiva

Day 60 opinion

Action: For adoption

Other

2.3.24. Birch pollen extract (*Betula verrucosa*) - EMEA-001879-PIP01-15-M02

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

Day 60 opinion

Action: For adoption

Pneumology - Allergology

2.3.25. Brexpiprazole - EMEA-001185-PIP01-11-M06

Otsuka Pharmaceutical Development & Commercialisation Europe GmbH; Treatment of schizophrenia

Day 60 opinion

Action: For adoption

Psychiatry

2.3.26. Dengue virus serotype 4 (live, attenuated) / Dengue virus serotype 3 (live, attenuated) / Dengue virus serotype 2 (live, attenuated) / Dengue virus serotype 1 (live, attenuated) - EMEA-001888-PIP01-15-M01

Takeda Vaccines, Inc.; Prevention of dengue fever

Day 60 opinion

Action: For adoption

Vaccines

2.4. Opinions on Re-examinations

2.5. Opinions on Review of Granted Waivers

No items

2.6. Finalisation and adoption of opinions

2.6.1. Vedolizumab - EMEA-000645-PIP01-09-M07

Takeda Pharma A/S; Crohn's Disease, Ulcerative colitis

Day 30 opinion

Action: For adoption

Gastroenterology-Hepatology

2.7. Partial Compliance Checks completed by EMA

2.7.1. Cemiplimab - EMEA-C1-002007-PIP02-17

Regeneron Ireland DAC.; Treatment of all conditions included in the category of malignant neoplasms (except haematopoietic and lymphoid tissue)

Day 30 letter

Action: For information

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

Genzyme Europe B.V.; Polycystic kidney, autosomal dominant / 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 90 discussion

Action: For discussion

Endocrinology-Gynaecology-Fertility-Metabolism / Uro-nephrology

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

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

Day 90 discussion

Action: For discussion

Gastroenterology-Hepatology

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

uniQure biopharma B.V.; Treatment of Haemophilia B

Day 90 discussion

Action: For discussion

Haematology-Hemostaseology

3.1.4. Olinciguat - EMEA-002759-PIP01-19

Treatment of sickle cell disease (SCD)

Day 90 discussion

Action: For discussion

Haematology-Hemostaseology

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

Synteract GmbH; sickle cell disease

Day 90 discussion

Action: For discussion

Haematology-Hemostaseology

3.1.6. EMEA-002742-PIP01-19

Treatment of parainfluenzae viral infection

Day 90 discussion

Action: For discussion

Infectious Diseases

3.1.7. EMEA-002755-PIP01-19

Prevention of lower respiratory tract infection caused by respiratory syncytial virus

Day 90 discussion

Action: For discussion

Infectious Diseases

3.1.8. 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 90 discussion

Action: For discussion

Infectious Diseases

3.1.9. EMEA-002635-PIP01-19

Treatment of advanced or metastatic malignancies harbouring anaplastic lymphoma kinase

ALK, ROS1, or NTRK1-3 alterations

Day 90 discussion

Action: For discussion

Oncology

3.1.10. 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 90 discussion

Action: For discussion

Oncology

3.1.11. 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 90 discussion

Action: For discussion

Oncology

3.1.12. EMEA-002814-PIP01-20

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

Day 90 discussion

Action: For discussion

Vaccines

3.1.13. Ralinepag - Orphan - EMEA-002432-PIP02-20

United Therapeutics Corporation; Treatment of pulmonary arterial hypertension / Treatment of pulmonary arterial hypertension WHO Group I to improve exercise capacity and to delay clinical worsening

Day 60 discussion

Action: For discussion

Cardiovascular Diseases

3.1.14. Sotatercept - EMEA-002756-PIP01-19

Pulmonary arterial hypertension

Day 60 discussion

Action: For discussion

Cardiovascular Diseases

3.1.15. Indometacin / Levonorgestrel - EMEA-002820-PIP01-20

Contraceptive management

Day 60 discussion

Action: For discussion

Endocrinology-Gynaecology-Fertility-Metabolism

3.1.16. Pioglitazone hydrochloride / Spironolactone / Metformin hydrochloride - EMEA-002187-PIP01-17

Treatment of polycystic ovary syndrome (PCOS) / Treatment of adolescent polycystic ovary syndrome (PCOS) in post-menarche adolescents <18 yrs and young adult women ≥18 yrs and <24.0 yrs

Day 60 discussion

Action: For discussion

Endocrinology-Gynaecology-Fertility-Metabolism

3.1.17. EMEA-002845-PIP01-20

Treatment of mucopolysaccharidosis II (Hunter syndrome) / Treatment of mucopolysaccharidosis II (MPS II, Hunter syndrome)

Day 60 discussion

Action: For discussion

Endocrinology-Gynaecology-Fertility-Metabolism

3.1.18. Maralixibat Chloride - EMEA-001475-PIP04-20

Biliary atresia (BA) / Treatment of Biliary atresia

Day 60 discussion

Action: For discussion

Gastroenterology-Hepatology

3.1.19. Recombinant monoclonal antibody to sialic acid-binding Ig-like lectin 8 - Orphan - EMEA-002856-PIP01-20

Allakos Inc; Treatment of eosinophilic gastrointestinal inflammatory disorders / Treatment of eosinophilic esophagitis / Treatment of eosinophilic gastritis and/or eosinophilic gastroenteritis

Day 60 discussion

Action: For discussion

Gastroenterology-Hepatology

3.1.20. Pegfilgrastim - EMEA-002671-PIP02-20

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

Day 60 discussion

Action: For discussion

Haematology-Hemostaseology

3.1.21. EMEA-002350-PIP03-20

Treatment of systemic lupus erythematosus (SLE) / Treatment of lupus nephritis (LN) despite receiving SoC, Treatment of systemic lupus erythematosus (SLE) despite receiving standard of care (SoC)

Day 60 discussion

Action: For discussion

Immunology-Rheumatology-Transplantation

3.1.22. Iscalimab - EMEA-002842-PIP01-20

Prophylaxis against transplant rejection / Prophylaxis of graft rejection in paediatric renal transplant patients 2 years of age and older

Day 60 discussion

Action: For discussion

Immunology-Rheumatology-Transplantation

3.1.23. Rimegepant - EMEA-002812-PIP02-20

Acute treatment of migraine

Day 60 discussion

Action: For discussion

Neurology

3.1.24. Viltolarsen - Orphan - EMEA-002853-PIP01-20

NS Pharma, Inc.; Treatment of Duchenne muscular dystrophy (DMD) / Treatment of Duchenne muscular dystrophy in patients who have a confirmed mutation of the DMD gene that is amenable to exon 53 skipping

Day 60 discussion

Action: For discussion

Neurology

3.1.25. Talazoparib - EMEA-002066-PIP01-20

Treatment of Ewing sarcoma / Treatment of refractory or recurrent Ewing Sarcoma

Day 60 discussion

Action: For discussion

Oncology

3.1.26. Flotetuzumab - EMEA-002855-PIP01-20

acute myeloid leukemia (AML)

Day 60 discussion

Action: For discussion

Oncology / Haematology-Hemostaseology

3.1.27. Atropine Sulfate - EMEA-002858-PIP01-20

Prevention of myopic progression

Day 60 discussion

Action: For discussion

Ophthalmology

3.1.28. Udonitrectag lysine - Orphan - EMEA-002848-PIP01-20

Recordati Rare Diseases; neurotrophic keratitis

Day 60 discussion

Action: For discussion

Ophthalmology

3.1.29. Garetosmab - Orphan - EMEA-002736-PIP01-19

Regeneron Ireland DAC; Treatment of Fibrodysplasia Ossificans Progressiva

Day 60 discussion

Action: For discussion

Other

3.1.30. Ravulizumab - EMEA-001943-PIP02-20

Hematopoietic Stem Cell Transplantation associated-Thrombotic Microangiopathy (HSCT-TMA) / Treatment of Thrombotic Microangiopathy after Haematopoietic Stem Cell Transplantation (HSCT-TMA)

Day 60 discussion

Action: For discussion

Uro-nephrology / Haematology-Hemostaseology

3.1.31. Multivalent pneumococcal polysaccharide conjugate to carrier protein - EMEA-002780-PIP02-20

Prevention of disease caused by *Streptococcus pneumoniae*

Day 60 discussion

Action: For discussion

Vaccines

3.1.32. EMEA-002870-PIP01-20

Induction of general anaesthesia in adults and children

Day 30 discussion

Action: For discussion

Anaesthesiology

3.1.33. Bisoprolol fumarate / ramipril - EMEA-002860-PIP01-20

Essential hypertension

Day 30 discussion

Action: For discussion

Cardiovascular Diseases

3.1.34. Dapagliflozin - EMEA-000694-PIP06-20

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

Day 30 discussion

Action: For discussion

Cardiovascular Diseases

3.1.35. Ziltivekimab - EMEA-002840-PIP01-20

Prevention of cardiovascular events in patients with atherosclerosis

Day 30 discussion

Action: For discussion

Cardiovascular Diseases

3.1.36. 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 30 discussion

Action: For discussion

Dermatology

3.1.37. Infigratinib - EMEA-002594-PIP02-20

Achondroplasia

Day 30 discussion

Action: For discussion

Endocrinology-Gynaecology-Fertility-Metabolism

3.1.38. Calmangfodipir - EMEA-002865-PIP01-20

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

Day 30 discussion

Action: For discussion

Gastroenterology-Hepatology

3.1.39. Lanifibranor - EMEA-002872-PIP01-20

NASH / Treatment of NASH

Day 30 discussion

Action: For discussion

Gastroenterology-Hepatology

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

Albireo AB; Alagille syndrome

Day 30 discussion

Action: For discussion

Gastroenterology-Hepatology

3.1.41. Vedolizumab - EMEA-000645-PIP04-20

Pouchitis (in patients who underwent proctocolectomy and ileal-pouch anal anastomosis for ulcerative colitis)

Day 30 discussion

Action: For discussion

Gastroenterology-Hepatology

3.1.42. EMEA-002863-PIP01-20

Paroxysmal Nocturnal Haemoglobinuria

Day 30 discussion

Action: For discussion

Haematology-Hemostaseology

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

Roche Registration GmbH; Treatment of membranous glomerulonephritis also known as membranous nephropathy (MN)

Day 30 discussion

Action: For discussion

Immunology-Rheumatology-Transplantation

3.1.44. EMEA-002866-PIP01-20

Treatment of idiopathic Parkinson's disease (PD)

Day 30 discussion

Action: For discussion

Neurology

3.1.45. [Tauroursodeoxycholic acid / Sodium phenylbutyrate - Orphan - EMEA-002876-PIP01-20](#)

Drug Development and Regulation SL; Treatment of Amyotrophic Lateral Sclerosis

Day 30 discussion

Action: For discussion

Neurology

3.1.46. [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 30 discussion

Action: For discussion

Oncology

3.1.47. [Delolimogene mupadenorepvec - Orphan - EMEA-002864-PIP01-20](#)

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

Day 30 discussion

Action: For discussion

Oncology

3.1.48. [Guadecitabine - Orphan - EMEA-001730-PIP03-20](#)

Otsuka Pharmaceutical B.V.; Myelodysplastic syndrome / Treatment of paediatric subjects age >3 months to <18 years with MDS or juvenile myelomonocytic leukemia (JMML) who are not candidates for haematopoietic cell transplant (HCT) (e.g. due to the absence of a suitable donor or other clinical reason) or subjects with relapsed MDS or JMML following treatment with HCT, Treatment of patients with intermediate or high risk MDS or chronic myeloid leukemia (CMML) whose disease progressed, relapsed or was refractory to hypomethylating agent (HMA) treatment

Day 30 discussion

Action: For discussion

Oncology

3.1.49. [Magrolimab - Orphan - EMEA-002819-PIP01-20](#)

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

Day 30 discussion

Action: For discussion

Oncology

3.1.50. [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 30 discussion

Action: For discussion

Oncology

3.1.51. [Tipifarnib - EMEA-002871-PIP01-20](#)

Treatment of HRAS mutant head and neck squamous cell carcinoma

Day 30 discussion

Action: For discussion

Oncology

3.1.52. [Doconexent \(Docosahexaenoic acid, DHA\) - Orphan - EMEA-002808-PIP01-20](#)

Natac Pharma S.L.; Retinitis Pigmentosa

Day 30 discussion

Action: For discussion

Ophthalmology

3.1.53. [\(2S,4S\)-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 30 discussion

Action: For discussion

Other / Haematology-Hemostaseology

[3.1.54. Autologous selected renal cells - EMEA-002844-PIP01-20](#)

Treatment of chronic kidney disease

Day 30 discussion

Action: For discussion

Uro-nephrology

[3.1.55. EMEA-002873-PIP01-20](#)

Active immunisation for the prevention of disease caused by chikungunya virus

Day 30 discussion

Action: For discussion

Vaccines

[3.1.56. EMEA-002862-PIP01-20](#)

Astra Zeneca; Prevention of COVID-19

Day 30 discussion

Action: For discussion

Article 7; PIP and Deferral and Waiver

Vaccines

[3.1.57. EMEA-002861-PIP02-20](#)

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

Day 30 discussion

Action: For discussion

Article 7; PIP and Deferral and Waiver

Vaccines

[3.1.58. EMEA-002597-PIP03-20](#)

Treatment of pemphigus

Day 30 discussion

Action: For discussion

Dermatology

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. Upadacitinib - EMEA-C1-001741-PIP04-17-M01

AbbVie Ltd; Treatment of atopic dermatitis

Day 30 discussion

Action: For discussion

Dermatology

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

Grifols Therapeutics LLC; Treatment of primary immunodeficiency

Day 30 discussion

Action: For discussion

Immunology-Rheumatology-Transplantation

3.2.3. Tecovirimat monohydrate - EMEA-C1-001205-PIP02-19

SiGA Technologies Inc; Orthopoxvirus disease (smallpox, monkeypox, cowpox, and vaccinia complications)

Day 30 discussion

Action: For discussion

Infectious Diseases

3.2.4. Inebilizumab - EMEA-C5-001911-PIP01-15-M02

Viela Bio; Treatment of neuromyelitis optica spectrum disorders

Day 30 discussion

Action: For discussion

Neurology

3.2.5. Iodine (¹³¹I) murine IgG1 monoclonal antibody against B7-H3 (¹³¹I-omburtamab) - EMEA-C1-002101-PIP02-18-M01

Y-mAbs Therapeutics A/S; Treatment of Neuroblastoma

Day 30 discussion

Action: For discussion

Oncology

3.2.6. [Lenadogene nolparvovec - EMEA-C1-001992-PIP02-16-M01](#)

GenSight Biologics; Treatment of Leber Hereditary Optic Neuropathy

Day 30 discussion

Action: For discussion

Ophthalmology

3.2.7. [Odevixibat - EMEA-C1-002054-PIP01-16-M02](#)

Albireo AB; Treatment of Progressive familial intrahepatic cholestasis (PFIC)

Day 30 discussion

Action: For discussion

Gastroenterology-Hepatology

3.2.8. [Anifrolumab - EMEA-C1-001435-PIP02-16-M01](#)

AstraZeneca AB; Treatment of systemic lupus erythematosus

Day 30 discussion

Action: For discussion

Immunology-Rheumatology-Transplantation

3.3. **Discussions on Modification of an Agreed Paediatric Investigation Plan**

3.3.1. [Captopril - EMEA-001544-PIP01-13-M02](#)

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

Day 30 discussion

Action: For discussion

Cardiovascular Diseases

3.3.2. [Landiolol \(hydrochloride\) - EMEA-001150-PIP02-13-M03](#)

AOP Orphan Pharmaceuticals AG; Treatment of supraventricular arrhythmias

Day 30 discussion

Action: For discussion

Cardiovascular Diseases

3.3.3. 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 30 discussion

Action: For discussion

Diagnostic / Oncology

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

AstraZeneca AB; Treatment of Type 2 Diabetes Mellitus

Day 30 discussion

Action: For discussion

Endocrinology-Gynaecology-Fertility-Metabolism

3.3.5. Ladarixin - EMEA-002642-PIP01-19-M02

Dompé farmaceutici S.p.A; Treatment of type 1 diabetes / Treatment of new-onset type 1 diabetes mellitus

Day 30 discussion

Action: For discussion

Endocrinology-Gynaecology-Fertility-Metabolism

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

Acerus Biopharma Inc.; Male hypogonadism

Day 30 discussion

Action: For discussion

Endocrinology-Gynaecology-Fertility-Metabolism

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

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

Day 30 discussion

Action: For discussion

Gastroenterology-Hepatology

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

Dova Pharmaceuticals Ireland Limited; Chemotherapy-induced thrombocytopenia /

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

Day 30 discussion

Action: For discussion

Haematology-Hemostaseology

3.3.9. [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 30 discussion

Action: For discussion

Haematology-Hemostaseology

3.3.10. [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 30 discussion

Action: For discussion

Infectious Diseases

3.3.11. [Cobicistat / atazanavir sulphate - EMEA-001465-PIP01-13-M03](#)

Bristol-Myers Squibb Pharma EEIG; Treatment of HIV-1 infection / in 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 30 discussion

Action: For discussion

Infectious Diseases

3.3.12. [Peramivir - EMEA-001856-PIP02-16-M01](#)

Biocryst Ireland Limited; Treatment of influenza

Day 30 discussion

Action: For discussion

Infectious Diseases

3.3.13. 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 30 discussion

Action: For discussion

Neurology

3.3.14. Inebilizumab - Orphan - EMEA-001911-PIP01-15-M03

Viela Bio, Inc; Neuromyelitis optica spectrum disorders

Day 30 discussion

Action: For discussion

Neurology

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

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

Day 30 discussion

Action: For discussion

Neurology

3.3.16. 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, haematopoietic and lymphoid tissue neoplasms) / 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 30 discussion

Action: For discussion

Oncology

3.3.17. 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 30 discussion

Action: For discussion

Oncology

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

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

Day 30 discussion

Action: For discussion

Other

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

Vertex Pharmaceuticals (Europe) Ltd; Cystic Fibrosis / Treatment of Cystic Fibrosis

Day 30 discussion

Action: For discussion

Other

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

Chiesi Farmaceutici S.p.A.; Cystic fibrosis

Day 30 discussion

Action: For discussion

Pneumology - Allergology

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

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

Day 30 discussion

Action: For discussion

Uro-nephrology

3.3.22. 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 30 discussion

Action: For discussion

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 13 October 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. EMEA-08-2020

Verona Pharma plc; All classes of medicinal products for treatment of chronic obstructive pulmonary disease (COPD) (excluding chronic lung diseases associated with long-term airflow limitation, such as asthma, bronchopulmonary dysplasia, primary cilia dyskinesia, obstructive lung disease related to graftversus-host disease after [bone-marrow] transplantation / Maintenance treatment of chronic obstructive pulmonary 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

None

8. Annual reports on deferrals

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

9. Organisational, regulatory and methodological matters

9.1. Mandate and organisation of the PDCO

None

9.2. Coordination with EMA Scientific Committees or CMDh-v

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

Action: For information

9.2.2. Interactions between CAT and PDCO

Outcome of the brainstorming meeting of 17 September 2020

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

9.4.1. European Network of Paediatric Research (Enpr) - European Medicines Agency (EMA)

Report from annual meeting of the members and Coordinating Group of the European network of paediatric research at the EMA (Enpr-EMA)

Action: For information

9.5. Cooperation with International Regulators

9.5.1. FDA cluster teleconference

Action: For information

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

None

9.6.1. Multi-regional clinical trials project on global paediatric trials

Action: For information

9.7. PDCO work plan

None

9.8. Planning and reporting

None

10. Any other business

10.1. Covid-19 update

Action: For information

10.2. Disclosure of confidential information - refresher training

Action: For information

11. Breakout sessions

11.1. Paediatric oncology

Action: For discussion on Tuesday 12:00-13:00

11.2. Neonatology

Action: For discussion on Tuesday 12:00 – 13:00

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/