



EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

9 December 2022
EMA/PDCO/898908/2022
Human Medicines Division

Paediatric Committee (PDCO)

Draft Agenda for the meeting on 13-16 December 2022

Chair: Brian Aylward – Vice-Chair: Sylvie Benchetrit

13 December 2022, 14:00 - 19:30, room 1D / virtual meeting

14 December 2022, 08:30 - 19:30, room 1D / virtual meeting

15 December 2022, 08:30 - 19:30, room 1D / virtual meeting

16 December 2022, 08:30 - 13:00, room 1D / 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 December 2022. See December 2022 PDCO minutes (to be published post January 2023 PDCO meeting).

1.2. Adoption of agenda

PDCO agenda for 13-16 December 2022.

1.3. Adoption of the minutes

PDCO minutes for 08-11 November 2022.

2. Opinions

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

2.1. Opinions on Products

2.1.1. Asundexian - EMEA-003144-PIP01-21

Prevention of arterial thromboembolism

Day 120 opinion

Action: For adoption

Cardiovascular Diseases

2.1.2. A 2'-MOE antisense oligonucleotide targeting apoC-III - EMEA-003177-PIP01-21

Treatment of familial chylomicronaemia syndrome

Day 120 opinion

Action: For adoption

Cardiovascular Diseases

2.1.3. Perflubutane - EMEA-003037-PIP02-22

Diagnostic evaluation of focal hepatic lesions

Day 120 opinion

Action: For adoption

Diagnostic / Oncology

2.1.4. Avexitide acetate - Orphan - EMEA-003125-PIP02-21

EigerBio Europe Limited; Treatment of congenital hyperinsulinism

Day 120 opinion

Action: For adoption

Endocrinology-Gynaecology-Fertility-Metabolism

2.1.5. Triheptanoin - Orphan - EMEA-001920-PIP04-19

Ultragenyx Germany GmbH; Treatment of long-chain fatty acid oxidation disorders (LC-FAOD)

Day 120 opinion

Action: For adoption

Endocrinology-Gynaecology-Fertility-Metabolism

2.1.6. Depemokimab - EMEA-003051-PIP05-22

Treatment of hypereosinophilic syndrome (HES)

Day 120 opinion

Action: For adoption

Haematology-Hemostaseology

2.1.7. Satralizumab - Orphan - EMEA-001625-PIP04-22

Roche Registration GmbH; Treatment of autoimmune encephalitis

Day 120 opinion

Action: For adoption

Neurology

2.1.8. Troriluzole (hydrochloride) - Orphan - EMEA-003084-PIP03-22

Biohaven Pharmaceutical Ireland DAC; Treatment of hereditary spinocerebellar ataxia

Day 120 opinion

Action: For adoption

Neurology

2.1.9. [Autologous CD4+ and CD8+ T cells transduced with lentiviral vector encoding a chimeric antigen receptor \(CAR\) directed against CD19 and preserving the T cell phenotype of the leukapheresis starting material - EMEA-003212-PIP01-22](#)

Treatment of mature B cell neoplasms

Day 120 opinion

Action: For adoption

Oncology

2.1.10. [Fianlimab - EMEA-003207-PIP01-22](#)

Treatment of melanoma

Day 120 opinion

Action: For adoption

Oncology

2.1.11. [Naxitamab - Orphan - EMEA-002346-PIP01-18](#)

Y-mAbs Therapeutics A/S; Short-term symptomatic treatment of pain / Treatment of neuroblastoma

Day 120 opinion

Action: For adoption

Oncology

2.1.12. [Tirzepatide - EMEA-002360-PIP02-22](#)

Treatment of obesity

Day 120 opinion

Action: For adoption

Other

2.1.13. [Landiolol - EMEA-001150-PIP03-22](#)

Treatment of ventricular arrhythmias

Day 60 opinion

Action: For adoption

Cardiovascular Diseases

2.1.14. Ziltivekimab - EMEA-002840-PIP02-22

Treatment of heart failure

Day 60 opinion

Action: For adoption

Cardiovascular Diseases

2.1.15. Efgartigimod alfa - EMEA-002597-PIP09-22

Treatment of bullous pemphigoid

Day 60 opinion

Action: For adoption

Dermatology

2.1.16. Setanaxib - Orphan - EMEA-003310-PIP01-22

Calliditas Therapeutics France SAS; Treatment of primary biliary cholangitis

Day 60 opinion

Action: For adoption

Gastroenterology-Hepatology

2.1.17. Acetylsalicylic acid / rivaroxaban - EMEA-003308-PIP01-22

Prevention of atherothrombotic events

Day 60 opinion

Action: For adoption

Haematology-Hemostaseology

2.1.18. Icerguastat - Orphan - EMEA-003312-PIP01-22

InFlectis BioScience S.A.S; Treatment of amyotrophic lateral sclerosis

Day 60 opinion

Action: For adoption

Neurology

2.1.19. Retifanlimab - Orphan - EMEA-002798-PIP03-22

Incyte Biosciences Distribution B.V.; Treatment of Merkel cell carcinoma

Day 60 opinion

Action: For adoption

Oncology

2.1.20. EMEA-003319-PIP01-22

Treatment of borderline personality disorder (BPD)

Day 60 opinion

Action: For adoption

Psychiatry

Note: Withdrawal request received on 29 November 2022

2.1.21. Tetanus toxoid - EMEA-003311-PIP01-22

Prevention of infectious disease caused by *Clostridium tetani*

Day 60 opinion

Action: For adoption

Vaccines

2.1.22. Live attenuated varicella virus - EMEA-003317-PIP01-22

Prevention of varicella

Day 60 opinion

Action: For adoption

Vaccines / Infectious Diseases

2.2. Opinions on Compliance Check

2.2.1. Ivacaftor [N-(2,4-Di-tert-butyl-5-hydroxyphenyl)-1,4-dihydro-4-oxoquinoline-3-carboxamide] - EMEA-C-000335-PIP01-08-M14

Vertex Pharmaceuticals (Ireland) Limited; Treatment of cystic fibrosis

Day 60 opinion

Action: For adoption

Other

Note: Withdrawal request received on 2 December 2022

2.2.2. Methylphenidate - EMEA-C-003189-PIP01-22

Laboratorios Lesvi S.L.; Treatment of attention-deficit hyperactivity disorder

Day 30 opinion

Action: For adoption

Neurology

2.3. Opinions on Modification of an Agreed Paediatric Investigation Plan

2.3.1. Macitentan - Orphan - EMEA-001032-PIP01-10-M05

Janssen-Cilag International NV; Treatment of systemic sclerosis / Treatment of pulmonary arterial hypertension / Treatment of idiopathic pulmonary fibrosis

Day 60 opinion

Action: For adoption

Cardiovascular Diseases

2.3.2. Human, recombinant, non-fucosylated IgG1k monoclonal antibody targeting OX-40 receptor on activated T cells - EMEA-002886-PIP01-20-M02

Amgen Europe B.V.; Treatment of atopic dermatitis

Day 60 opinion

Action: For adoption

Dermatology

2.3.3. Avatrombopag maleate - EMEA-001136-PIP01-11-M02

Swedish Orphan Biovitrum AB; Treatment of chronic immune thrombocytopenia

Day 60 opinion

Action: For adoption

Haematology-Hemostaseology

2.3.4. Crovalimab - EMEA-002709-PIP01-19-M01

Roche Registration GmbH; Treatment of paroxysmal nocturnal haemoglobinuria / Treatment of atypical haemolytic uremic syndrome

Day 60 opinion

Action: For adoption

Haematology-Hemostaseology

2.3.5. Garadacimab - Orphan - EMEA-002726-PIP01-19-M03

CSL Behring GmbH; Prevention of hereditary angioedema attacks

Day 60 opinion

Action: For adoption

Haematology-Hemostaseology

2.3.6. [Allogeneic bone marrow derived mesenchymal stromal cells, ex-vivo expanded - Orphan - EMEA-002706-PIP01-19-M02](#)

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

Day 60 opinion

Action: For adoption

Immunology-Rheumatology-Transplantation

2.3.7. [Avibactam / ceftazidime - EMEA-001313-PIP01-12-M13](#)

Pfizer Europe MA EEIG; Treatment of bacterial infections

Day 60 opinion

Action: For adoption

Infectious Diseases

2.3.8. [Aztreonam / avibactam - EMEA-002283-PIP01-17-M04](#)

Pfizer Europe MA EEIG; Treatment of infections caused by aerobic gram-negative bacteria

Day 60 opinion

Action: For adoption

Infectious Diseases

2.3.9. [Cobicistat / atazanavir sulphate - EMEA-001465-PIP01-13-M05](#)

Bristol-Myers Squibb Pharma EEIG; Treatment of human immunodeficiency virus (HIV-1) infection

Day 60 opinion

Action: For adoption

Infectious Diseases

2.3.10. [Cobicistat / darunavir - EMEA-001280-PIP01-12-M05](#)

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

Day 60 opinion

Action: For adoption

Infectious Diseases

2.3.11. [Lamivudine \(3TC\) / abacavir \(ABC\) / dolutegravir \(DTG\) - EMEA-001219-PIP01-11-M06](#)

ViiV Healthcare UK Limited; Treatment of human immunodeficiency virus (HIV-1) infection

Day 60 opinion

Action: For adoption

Infectious Diseases

2.3.12. [Tazobactam / ceftolozane - EMEA-001142-PIP02-16-M01](#)

Merck Sharp & Dohme (Europe), Inc.; Treatment of pneumonia

Day 60 opinion

Action: For adoption

Infectious Diseases

2.3.13. [Tenofovir alafenamide / emtricitabine / cobicistat / darunavir - EMEA-001825-PIP01-15-M04](#)

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

Day 60 opinion

Action: For adoption

Infectious Diseases

2.3.14. [Isoflurane - EMEA-002320-PIP01-17-M03](#)

Sedana Medical AB; Sedation of mechanically ventilated patients

Day 60 opinion

Action: For adoption

Neonatology - Paediatric Intensive Care

2.3.15. [Fordadistrogene movaparvovec - Orphan - EMEA-002741-PIP01-20-M01](#)

Pfizer Europe MA EEIG; Treatment of Duchenne muscular dystrophy

Day 60 opinion

Action: For adoption

Neurology

2.3.16. Galcanezumab - EMEA-001860-PIP03-16-M08

Eli Lilly and Company Limited; Prevention of migraine headache

Day 60 opinion

Action: For adoption

Neurology

2.3.17. Evoncabtagene pazurgedleucel - EMEA-002881-PIP01-20-M01

CRISPR Therapeutics AG; Treatment of B-lymphoblastic leukaemia/lymphoma / Treatment of mature B cell neoplasms

Day 60 opinion

Action: For adoption

Oncology

2.3.18. Calcium chloride / aprotinin / thrombin / fibrinogen - EMEA-001079-PIP01-10-M06

Kedrion S.p.A.; Treatment of haemorrhage resulting from a surgical procedure / Prevention of haemorrhage resulting from a surgical procedure

Day 60 opinion

Action: For adoption

Other

2.3.19. Lanadelumab - Orphan - EMEA-001864-PIP03-19-M01

Takeda Pharmaceuticals International AG Ireland Branch; Prevention of attacks of idiopathic non-histaminergic angioedema (INHA)

Day 60 opinion

Action: For adoption

Other

2.3.20. Azelastine hydrochloride / mometasone furoate - EMEA-003122-PIP01-21-M01

Lek Pharmaceuticals d.d.; Treatment of seasonal allergic rhinitis

Day 60 opinion

Action: For adoption

Oto-rhino-laryngology

2.3.21. Zuranolone - EMEA-003119-PIP01-21-M01

Biogen Netherlands B.V.; Treatment of postpartum depression

Day 60 opinion

Action: For adoption

Psychiatry

2.3.22. Mirabegron - EMEA-000597-PIP02-10-M09

Astellas Pharma Europe B.V.; Treatment of idiopathic overactive bladder

Day 60 opinion

Action: For adoption

Uro-nephrology

2.3.23. Synthetic double-stranded siRNA oligonucleotide directed against lactate dehydrogenase A mRNA and containing four modified nucleosides which form a ligand cluster of four N-acetylgalactosamine residues (DCR-PHXC, nedosiran) - Orphan - EMEA-002493-PIP01-18-M05

Dicerna Ireland Limited; Treatment of primary hyperoxaluria

Day 60 opinion

Action: For adoption

Uro-nephrology

2.3.24. *Neisseria meningitidis* serogroup B fHbp subfamily B / *Neisseria meningitidis* serogroup B fHbp subfamily A / *Neisseria meningitidis* group Y polysaccharide conjugated to tetanus toxoid carrier protein / *Neisseria meningitidis* group W-135 polysaccharide conjugated to tetanus toxoid carrier protein / *Neisseria meningitidis* group C polysaccharide conjugated to tetanus toxoid carrier protein / *Neisseria meningitidis* group A polysaccharide conjugated to tetanus toxoid carrier protein - EMEA-002814-PIP02-21-M01

Pfizer Europe MA EEIG; Invasive disease caused by *Neisseria meningitidis* group A, B, C, W and Y from 12 months of age to less than 18 years of age

Day 60 opinion

Action: For adoption

Vaccines

2.3.25. SARS-CoV-2 virus, beta-propiolactone inactivated adjuvanted with CpG 1018 (VLA2001) - EMEA-003077-PIP01-21-M01

Valneva Austria GmbH; Prevention of coronavirus disease 2019 (COVID-19)

Day 60 opinion

Action: For adoption

Vaccines

2.3.26. [Ivacaftor - EMEA-000335-PIP01-08-M15](#)

Vertex Pharmaceuticals (Ireland) Ltd; Treatment of cystic fibrosis

Day 30 opinion

Action: for adoption

Other

2.4. **Opinions on Re-examinations**

No item

2.5. **Opinions on Review of Granted Waivers**

No item

2.6. **Finalisation and adoption of Opinions**

No item

2.7. **Partial Compliance Checks completed by EMA**

The following partial compliance checks have been identified by the PME coordinator and PDCO rapporteur as not needing to be referred to the PDCO for discussion. The PDCO has been informed in writing.

2.7.1. [Concizumab - EMEA-C1-002326-PIP04-20](#)

Novo Nordisk A/S; Treatment of congenital haemophilia A

Day 30 letter

Action: For information

Haematology-Hemostaseology

2.7.2. [Odevixibat - EMEA-C1-002054-PIP03-20-M02](#)

Albireo AB; Treatment of Alagille syndrome

Day 30 letter

Action: For information

Gastroenterology-Hepatology

2.7.3. [Bosutinib - EMEA-C1-000727-PIP01-09-M06](#)

Pfizer Europe MA EEIG; Treatment of chronic myeloid leukaemia

Day 30 letter

Action: For information

Oncology

3. Discussion of applications

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

3.1. Discussions on Products D90-D60-D30

3.1.1. [Zilebesiran - EMEA-003218-PIP01-22](#)

Treatment of hypertension

Day 90 discussion

Action: For discussion

Cardiovascular Diseases

3.1.2. [Danuglipron - EMEA-002944-PIP02-22](#)

Treatment of chronic weight management/obesity / Treatment of obesity

Day 90 discussion

Action: For discussion

Endocrinology-Gynaecology-Fertility-Metabolism

3.1.3. [Insulin lispro - EMEA-003166-PIP01-21](#)

Treatment of diabetes mellitus type 1 / Treatment of diabetes mellitus type 2

Day 90 discussion

Action: For discussion

Endocrinology-Gynaecology-Fertility-Metabolism

3.1.4. [EMEA-003241-PIP01-22](#)

Treatment of sickle cell disease

Day 90 discussion

Action: For discussion

Haematology-Hemostaseology

3.1.5. [Adeno-associated virus serotype hu68 containing the human GLB1 gene - Orphan - EMEA-003102-PIP01-21](#)

Passage Bio, Inc.; Treatment of GM1 gangliosidosis

Day 90 discussion

Action: For discussion

Neurology

3.1.6. [Cemdisiran \(sodium\) - Orphan - EMEA-003237-PIP01-22](#)

Regeneron Ireland DAC; Treatment of myasthenia gravis

Day 90 discussion

Action: For discussion

Neurology

3.1.7. [Pozelimab - EMEA-003238-PIP01-22](#)

Treatment of myasthenia gravis

Day 90 discussion

Action: For discussion

Neurology

3.1.8. [Lutetium \(177Lu\) edotreotide - Orphan - EMEA-003245-PIP01-22](#)

ITM Solucin GmbH; Treatment of gastro-entero-pancreatic neuroendocrine tumours (GEP-NETs)

Day 90 discussion

Action: For discussion

Oncology

3.1.9. [Obecabtagene autoleucel - Orphan - EMEA-003171-PIP01-21](#)

Autolus GmbH; Treatment of acute lymphoblastic leukaemia

Day 90 discussion

Action: For discussion

Oncology

3.1.10. Humanised IgG2 monoclonal antibody against interleukin-6 - EMEA-003215-PIP01-22

Treatment of macular oedema

Day 90 discussion

Action: For discussion

Ophthalmology

3.1.11. Furosemide - EMEA-003316-PIP01-22

Treatment of fluid retention

Day 60 discussion

Action: For discussion

Cardiovascular Diseases

3.1.12. Barzolvolimab - EMEA-003327-PIP01-22

Treatment of chronic spontaneous urticaria

Day 60 discussion

Action: For discussion

Dermatology

3.1.13. Povorcitinib - EMEA-003313-PIP01-22

Treatment of hidradenitis suppurativa

Day 60 discussion

Action: For discussion

Dermatology

3.1.14. Rilzabrutinib - EMEA-002438-PIP03-22

Treatment of atopic dermatitis

Day 60 discussion

Action: For discussion

Dermatology

3.1.15. Ritlecitinib - EMEA-002451-PIP03-22

Treatment of vitiligo

Day 60 discussion

Action: For discussion

Dermatology

3.1.16. Upadacitinib - EMEA-001741-PIP07-22

Treatment of vitiligo

Day 60 discussion

Action: For discussion

Dermatology

3.1.17. Recombinant human glutamic acid dextranoylase (rhGAD65) - EMEA-000609-PIP02-22

Prevention or delay of clinical type 1 diabetes mellitus / Prevention of type 1 diabetes mellitus

Day 60 discussion

Action: For discussion

Endocrinology-Gynaecology-Fertility-Metabolism

3.1.18. Ciraparantag - EMEA-003321-PIP01-22

Treatment of FXa inhibitor-associated haemorrhage / Prevention of FXa inhibitor-associated haemorrhage

Day 60 discussion

Action: For discussion

Haematology-Hemostaseology

3.1.19. Izokibep - EMEA-003325-PIP01-22

Treatment of chronic idiopathic arthritis (including rheumatoid arthritis, ankylosing spondylarthritis, psoriatic arthritis, and juvenile idiopathic arthritis)

Day 60 discussion

Action: For discussion

Immunology-Rheumatology-Transplantation

3.1.20. EMEA-003326-PIP01-22

Treatment of infections caused by gram-negative organisms / Complicated urinary tract infections (cUTI) / Hospital associated pneumonia or ventilator associated pneumonia

Day 60 discussion

Action: For discussion

Infectious Diseases

[3.1.21. Lenacapavir / bictegravir - EMEA-003324-PIP01-22](#)

Treatment of human immunodeficiency virus (HIV-1) infection

Day 60 discussion

Action: For discussion

Infectious Diseases

[3.1.22. Luminol - EMEA-003322-PIP01-22](#)

Treatment of coronavirus disease 2019 (COVID-19)

Day 60 discussion

Action: For discussion

Infectious Diseases

[3.1.23. Posoleucel - Orphan - EMEA-002908-PIP02-22](#)

Allovir International DAC; Prevention of viral disease in haematopoietic stem cell transplantation (HCT)

Day 60 discussion

Action: For discussion

Infectious Diseases

[3.1.24. RNA replicase inhibitor - EMEA-003306-PIP01-22](#)

Treatment of coronavirus disease 2019 (COVID-19)

Day 60 discussion

Action: For discussion

Infectious Diseases

[3.1.25. Uproleselan - Orphan - EMEA-003307-PIP01-22](#)

GlycoMimetics, Inc.; Treatment of acute myeloid leukaemia

Day 60 discussion

Action: For discussion

Oncology

3.1.26. [Vutrisiran - Orphan - EMEA-002425-PIP02-22](#)

Anylam Netherlands B.V.; Treatment of Stargardt disease

Day 60 discussion

Action: For discussion

Ophthalmology

3.1.27. [Dersimelagon - EMEA-002850-PIP03-22](#)

Treatment of systemic sclerosis

Day 60 discussion

Action: For discussion

Other

3.1.28. [mRNA encoding modified human ornithine transcarbamylase - Orphan - EMEA-003315-PIP01-22](#)

Arcturus Therapeutics Europe B.V.; Treatment of ornithine transcarbamylase deficiency / Treatment of ornithine transcarbamylase deficiency, which is characterised by episodes of hyperammonemia and consequent sequelae

Day 60 discussion

Action: For discussion

Other

3.1.29. [Dexpramipexole - EMEA-003328-PIP01-22](#)

Treatment of asthma

Day 60 discussion

Action: For discussion

Pneumology - Allergology

3.1.30. [Pamrevlumab - EMEA-002979-PIP04-22](#)

Treatment of interstitial lung diseases with fibrosis

Day 60 discussion

Action: For discussion

Pneumology - Allergology

3.1.31. EMEA-003319-PIP02-22

Treatment of major depressive disorder (MDD)

Day 60 discussion

Action: For discussion

Psychiatry

3.1.32. EMEA-003319-PIP03-22

Treatment of posttraumatic stress disorder (PTSD)

Day 60 discussion

Action: For discussion

Psychiatry

3.1.33. Influenza recombinant H7 haemagglutinin - EMEA-003314-PIP01-22

Prevention of influenza infection

Day 60 discussion

Action: For discussion

Vaccines

3.1.34. Phuket modRNA / Darwin modRNA / Austria modRNA / Wisconsin modRNA - EMEA-003318-PIP01-22

Prevention of influenza disease

Day 60 discussion

Action: For discussion

Vaccines

3.1.35. Single-stranded 5' capped mRNA encoding the respiratory syncytial virus glycoprotein F stabilised in the prefusion conformation - EMEA-003309-PIP01-22

Prevention of lower respiratory tract illness (LRTI) caused by respiratory syncytial virus (RSV)

Day 60 discussion

Action: For discussion

Vaccines

3.1.36. Fenofibrate / rosuvastatin - EMEA-003332-PIP01-22

Prevention of cardiovascular events / Treatment of mixed hyperlipidaemia

Day 30 discussion

Action: For discussion

Cardiovascular Diseases

3.1.37. [Hydrochlorothiazide / amlodipine / candesartan cilexetil - EMEA-002024-PIP02-22](#)

Treatment of hypertension

Day 30 discussion

Action: For discussion

Cardiovascular Diseases

3.1.38. [Clazakizumab - EMEA-001371-PIP03-22](#)

Prevention of cardiovascular events in patients with atherosclerosis undergoing dialysis

Day 30 discussion

Action: For discussion

Cardiovascular Diseases / Uro-nephrology

3.1.39. [Clascoterone - EMEA-003330-PIP01-22](#)

Treatment of acne vulgaris

Day 30 discussion

Action: For discussion

Dermatology

3.1.40. [Encaleret - Orphan - EMEA-003348-PIP01-22](#)

Calcilytix Therapeutics, Inc a BridgeBio Company; Treatment of hypoparathyroidism

Day 30 discussion

Action: For discussion

Endocrinology-Gynaecology-Fertility-Metabolism

3.1.41. [Recombinant human tissue nonspecific alkaline phosphatase \(TNSALP\) fragment crystallizable \(Fc\) deca aspartate fusion protein - EMEA-003343-PIP01-22](#)

Treatment of hypophosphatasia

Day 30 discussion

Action: For discussion

Endocrinology-Gynaecology-Fertility-Metabolism

3.1.42. [Sodium\(4-{{E}}-3-\(4-fluorophenyl\)-3-\[4-\(3-morpholin-4-yl-prop-1-ynyl\)phenyl\]allyloxy}-2-methylphenoxy\)acetate - Orphan - EMEA-003331-PIP01-22](#)

Reneo Pharmaceuticals Inc; Treatment of primary mitochondrial disorders

Day 30 discussion

Action: For discussion

Endocrinology-Gynaecology-Fertility-Metabolism

3.1.43. [EMEA-003002-PIP03-22](#)

Treatment of portal hypertension with compensated cirrhosis

Day 30 discussion

Action: For discussion

Gastroenterology-Hepatology

3.1.44. [EMEA-003090-PIP02-22](#)

Treatment of hereditary angioedema

Day 30 discussion

Action: For discussion

Haematology-Hemostaseology

3.1.45. [Enpatoran - EMEA-003342-PIP02-22](#)

Treatment of systemic lupus erythematosus

Day 30 discussion

Action: For discussion

Immunology-Rheumatology-Transplantation

3.1.46. [Enpatoran - EMEA-003342-PIP01-22](#)

Treatment of cutaneous lupus erythematosus

Day 30 discussion

Action: For discussion

Immunology-Rheumatology-Transplantation / Dermatology

3.1.47. [Trimodulin \(human IgM, IgA, IgG solution\) - EMEA-002883-PIP02-22](#)

Treatment of coronavirus disease 2019 (COVID-19)

Day 30 discussion

Action: For discussion

Neonatology - Paediatric Intensive Care / Infectious Diseases

3.1.48. Recombinant human arylsulfatase A - Orphan - EMEA-002050-PIP02-22

Takeda Pharmaceuticals International AG Ireland Branch; Treatment of metachromatic leukodystrophy

Day 30 discussion

Action: For discussion

Neurology

3.1.49. Sodium ({{(2S)-1,4-bis[2-(4-chloro-3-fluorophenoxy)acetamido]bicyclo[2.2.2]octan-2-yl}oxy)methyl hydrogen phosphate-2-amino-2-(hydroxymethyl)propane-1,3-diol (1/1/1) - Orphan - EMEA-003344-PIP01-22

Calico Life Sciences LLC; Treatment of vanishing white matter disease

Day 30 discussion

Action: For discussion

Neurology

3.1.50. Eftilagimod alpha - EMEA-002698-PIP02-22

Treatment of head and neck squamous cell carcinoma (HNSCC)

Day 30 discussion

Action: For discussion

Oncology

3.1.51. Eftilagimod alpha - EMEA-002698-PIP03-22

Treatment of non-small cell lung cancer

Day 30 discussion

Action: For discussion

Oncology

3.1.52. Pembrolizumab - EMEA-001474-PIP03-22

Treatment of Hodgkin lymphoma / Treatment of all conditions included in the category of malignant neoplasms (except nervous system, haematopoietic and lymphoid tissue)

Day 30 discussion

Action: For discussion

Oncology

3.1.53. Sotorasib - EMEA-002690-PIP02-22

Treatment of malignant colorectal neoplasms

Day 30 discussion

Action: For discussion

Oncology

3.1.54. Upifitamab rilsodotin - EMEA-003340-PIP01-22

Treatment of primary peritoneal cancer / Treatment of fallopian tube cancer / Treatment of ovarian cancer

Day 30 discussion

Action: For discussion

Oncology

3.1.55. An acellular tubular graft composed of human collagen types I and III and other extracellular matrix proteins, including fibronectin and vitronectin - EMEA-003345-PIP01-22

Treatment of vascular injuries

Day 30 discussion

Action: For discussion

Other

3.1.56. Hemopexin, human - Orphan - EMEA-003333-PIP01-22

CSL Behring GmbH; Treatment of sickle cell disease

Day 30 discussion

Action: For discussion

Other

3.1.57. Cyanocobalamin / pyridoxine / thiamine / diclofenac - EMEA-003292-PIP02-22

Treatment of inflammatory rheumatic diseases / Treatment of inflammatory pain

Day 30 discussion

Action: For discussion

Pain

3.1.58. Orphenadrine / diclofenac - EMEA-003337-PIP01-22

Treatment of acute painful muscle spasms / Treatment of acute pain and inflammation /
Treatment of musculoskeletal pain / Treatment of acute painful muscle spasms

Day 30 discussion

Action: For discussion

Pain

3.1.59. Thiocolchicoside / diclofenac - EMEA-003339-PIP01-22

Treatment of musculoskeletal pain / Treatment of acute painful muscle spasms

Day 30 discussion

Action: For discussion

Pain

3.1.60. Camlipixant - EMEA-003334-PIP01-22

Treatment of refractory chronic cough

Day 30 discussion

Action: For discussion

Pneumology - Allergology

3.1.61. EMEA-003347-PIP01-22

Treatment of steroid-resistant nephrotic syndrome

Day 30 discussion

Action: For discussion

Uro-nephrology

3.1.62. Single-stranded 5' capped mRNA encoding the HAs of the influenza virus - EMEA-003346-PIP01-22

Prevention of influenza disease

Day 30 discussion

Action: For discussion

Vaccines

3.1.63. Varicella / Rubella virus (live, attenuated) / Measles virus (live, attenuated) / Mumps virus (live, attenuated) - EMEA-003341-PIP01-22

Prevention of measles, mumps, rubella and varicella

Day 30 discussion

Action: For discussion

Vaccines / Infectious Diseases

3.2. Discussions on Compliance Check

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

3.2.1. Empagliflozin - EMEA-C-000828-PIP01-09-M09

Boehringer Ingelheim International GmbH; Treatment of type 2 diabetes mellitus

Day 30 discussion

Action: For discussion

Endocrinology-Gynaecology-Fertility-Metabolism

3.2.2. Linagliptin - EMEA-C-000498-PIP01-08-M10

Boehringer Ingelheim International GmbH; Treatment of type 2 diabetes mellitus

Day 30 discussion

Action: For discussion

Endocrinology-Gynaecology-Fertility-Metabolism

3.2.3. Avacopan - EMEA-C4-002023-PIP01-16-M06

ChemoCentryx Ireland Ltd.; Treatment of anti-neutrophil cytoplasmic auto-antibody (ANCA)-associated vasculitis

Day 30 discussion

Action: For discussion

Immunology-Rheumatology-Transplantation

3.2.4. Oteseconazole - EMEA-C-002392-PIP01-18-M02

Gedeon Richter Plc.; Treatment of vulvovaginal candidiasis

Day 30 discussion

Action: For discussion

Infectious Diseases

3.2.5. *Neisseria meningitidis* serogroup B fHbp subfamily B / *Neisseria meningitidis* serogroup B fHbp subfamily A / *Neisseria meningitidis* group A polysaccharide

conjugated to tetanus toxoid carrier protein / *Neisseria meningitidis* group W-135 polysaccharide conjugated to tetanus toxoid carrier protein / *Neisseria meningitidis* group Y polysaccharide conjugated to tetanus toxoid carrier protein / *Neisseria meningitidis* group C polysaccharide conjugated to tetanus toxoid carrier protein - EMEA-C1-002814-PIP02-21

Pfizer Europe MA EEIG; Treatment of invasive disease caused by *Neisseria meningitidis* group A, B, C, W and Y from 2 months of age

Day 30 discussion

Action: For discussion

Vaccines

3.2.6. Pandemic influenza vaccine (H5N1) (surface antigen, inactivated, adjuvanted) - EMEA-C-001830-PIP01-15-M02

Seqirus S.r.l.; Prevention of influenza infection

Day 30 discussion

Action: For discussion

Vaccines

3.2.7. Pneumococcal polysaccharide conjugate vaccine (20-valent, adsorbed) - EMEA-C3-002330-PIP01-18-M02

Pfizer Europe MA EEIG; Prevention of disease caused by *Streptococcus pneumoniae*

Day 30 discussion

Action: For discussion

Vaccines

3.3. Discussions on Modification of an Agreed Paediatric Investigation Plan

3.3.1. Crinecerfont; 2-Thiazolamine, 4-(2-chloro-4-methoxy-5-methylphenyl)-N-[(1S)-2-cyclopropyl-1-(3-fluoro-4-methylphenyl)ethyl]-5-methyl-N-2-propyn-1-yl; NBI-74788 - Orphan - EMEA-002700-PIP01-19-M01

Neurocrine Therapeutics Ltd.; Treatment of congenital adrenal hyperplasia

Day 30 discussion

Action: For discussion

Endocrinology-Gynaecology-Fertility-Metabolism

3.3.2. Drospirenone / estetrol monohydrate - EMEA-001332-PIP01-12-M06

Estetra SRL; Prevention of pregnancy

Day 30 discussion

Action: For discussion

Endocrinology-Gynaecology-Fertility-Metabolism

3.3.3. [Evinacumab - EMEA-002298-PIP01-17-M05](#)

Ultragenyx Germany GmbH; Treatment of elevated cholesterol

Day 30 discussion

Action: For discussion

Endocrinology-Gynaecology-Fertility-Metabolism

3.3.4. [Ibutamoren mesilate - Orphan - EMEA-003032-PIP01-21-M01](#)

Lumos Pharma, Inc.; Treatment of growth hormone deficiency

Day 30 discussion

Action: For discussion

Endocrinology-Gynaecology-Fertility-Metabolism

3.3.5. [Baricitinib - EMEA-001220-PIP01-11-M07](#)

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

Day 30 discussion

Action: For discussion

Immunology-Rheumatology-Transplantation

3.3.6. [Baloxavir marboxil - EMEA-002440-PIP01-18-M04](#)

Roche Registration GmbH; Treatment of influenza / Prevention of influenza

Day 30 discussion

Action: For discussion

Infectious Diseases

3.3.7. [Relebactam monohydrate / cilastatin sodium / imipenem monohydrate - EMEA-001809-PIP01-15-M03](#)

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

Day 30 discussion

Action: For discussion

Infectious Diseases

3.3.8. [Fremanezumab - EMEA-001877-PIP01-15-M03](#)

Teva GmbH; Prevention of migraine headaches

Day 30 discussion

Action: For discussion

Neurology

3.3.9. [Glycopyrronium bromide - EMEA-001366-PIP01-12-M03](#)

Proveca Pharma Limited; Treatment of sialorrhoea (chronic pathological drooling)

Day 30 discussion

Action: For discussion

Neurology

3.3.10. [Quizartinib - Orphan - EMEA-001821-PIP01-15-M06](#)

Daiichi Sankyo Europe GmbH; Treatment of acute myeloid leukaemia

Day 30 discussion

Action: For discussion

Oncology

3.3.11. [Gilteritinib \(as fumarate\) - Orphan - EMEA-002064-PIP01-16-M04](#)

Astellas Pharma Europe B.V.; Treatment of acute myeloid leukaemia

Day 30 discussion

Action: For discussion

Oncology / Haematology-Hemostaseology

3.3.12. [Inotuzumab ozogamicin - Orphan - EMEA-001429-PIP01-13-M07](#)

Pfizer Europe MA EEIG; Treatment of acute lymphoblastic leukaemia

Day 30 discussion

Action: For discussion

Oncology / Haematology-Hemostaseology

3.3.13. [Afamelanotide - Orphan - EMEA-000737-PIP02-11-M02](#)

Clinuvel Europe Limited; Treatment of erythropoietic protoporphyria

Day 30 discussion

Action: For discussion

Other

3.3.14. Selexipag - EMEA-000997-PIP01-10-M06

Janssen-Cilag International NV; Treatment of pulmonary arterial hypertension

Day 30 discussion

Action: For discussion

Other

3.3.15. Begelomab - Orphan - EMEA-001744-PIP01-14-M02

ADIENNE S.r.l SU; Treatment of acute graft-versus-host disease (aGvHD)

Day 30 discussion

Action: For discussion

Other / Immunology-Rheumatology-Transplantation

3.3.16. Methoxflurane - EMEA-000334-PIP01-08-M11

Medical Developments UK Ltd; Treatment of acute pain

Day 30 discussion

Action: For discussion

Pain

3.3.17. Budesonide / glycopyrronium bromide / formoterol fumarate dihydrate - EMEA-002063-PIP01-16-M02

AstraZeneca AB; Treatment of asthma

Day 30 discussion

Action: For discussion

Pneumology - Allergology

3.3.18. Cariprazine hydrochloride - EMEA-001652-PIP01-14-M04

Gedeon Richter Plc.; Treatment of schizophrenia

Day 30 discussion

Action: For discussion

Psychiatry

3.3.19. Lumasiran sodium - Orphan - EMEA-002079-PIP01-16-M03

Alnylam UK Limited; Treatment of primary hyperoxaluria type 1

Day 30 discussion

Action: For discussion

Uro-nephrology

4. Nominations

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

4.1. List of submissions of applications with start of procedure 3 January 2023 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

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

6. Discussion on the applicability of class waivers

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

6.1. Discussions on the applicability of class waiver for products

No item

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

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

No item

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. PDCO membership

Action: For information

9.1.2. Vote by Proxy

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 Party: D30 Products identified

PDCO member: Karen van Malderen

Action: For information

9.3.2. Formulation Working Group

Proposal to PDCO - PDCO Formulation Working Group experts for 2023

PDCO member: Brian Aylward (*ad interim*)

Action: For adoption

9.3.3. Modelling and Simulation Working Party (MSWP)

PDCO member: Kristin Karlsson

Action: For information

9.3.4. Methodology Working Party (MWP)

Introduction of MWP to the Committees

PDCO member: Kristin Karlsson; MWP Chair: Christian Roes

Action: For information

9.4. Cooperation within the EU regulatory network

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

Action: For information

9.5. Cooperation with International Regulators

9.5.1. Paediatric Cluster Teleconference

Action: For information

9.5.2. Food and Drug Administration (FDA) – Introduction Liaison Official

Action: For information

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

No item

9.7. PDCO work plan

9.7.1. Workplan for 2023

PDCO Chair: Brian Aylward

Action: For adoption

9.8. Planning and reporting

9.8.1. EMA Business Pipeline activity and Horizon scanning

Q4/2022 Update of the Business Pipeline report for the human scientific committees

Action: For information

10. Any other business

10.1. COVID-19 update

Action: For information

10.2. Upcoming Innovation Task Force (ITF) meetings

Action: For information

10.3. ICH M11 Public Consultation

Action: For information

11. Breakout sessions

11.1. HIV

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

11.2. Neonatology

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

11.3. Paediatric oncology

Action: For discussion on Wednesday, 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/