Paediatric Committee (PDCO)
Draft Agenda for the meeting on 23-26 April 2024

Chair: Brian Aylward – Vice-Chair: Sylvie Benchetrit

23 April 2024, 14:00 - 19:00, Virtual meeting
24 April 2024, 08:30 - 19:00, Virtual meeting
25 April 2024, 08:30 - 19:00, Virtual meeting
26 April 2024, 08:30 - 13:00, Virtual meeting

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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 23-26 April 2024. See April 2024 PDCO minutes (to be published post May 2024 PDCO meeting).

1.2. Adoption of agenda

PDCO agenda for 23-26 April 2024.

1.3. Adoption of the minutes

PDCO minutes for 19-22 March 2024.

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. EMEA-003480-PIP01-23

Treatment of psoriasis
Day 120 opinion
Action: For adoption
Dermatology

2.1.2. Zasocitinib - EMEA-003478-PIP01-23

Treatment of psoriasis
Day 120 opinion
Action: For adoption
Dermatology

2.1.3. Human alpha-1 proteinase inhibitor, modified - EMEA-003463-PIP01-23

Treatment of haemophilia B
Day 120 opinion

**Action**: For adoption

*Haematology-Hemostaseology*

### 2.1.4. Ianalumab - EMEA-002338-PIP05-23

Treatment of immune thrombocytopenia (ITP)

Day 120 opinion

**Action**: For adoption

*Haematology-Hemostaseology*

### 2.1.5. Mavorixafor - Orphan - EMEA-002490-PIP01-18

X4 Pharmaceuticals (Austria) GmbH; Treatment of warts, hypogammaglobulinemia, infections and myelokathexis (WHIM) syndrome

Day 120 opinion

**Action**: For adoption

*Immunology-Rheumatology-Transplantation*

### 2.1.6. Ganaxolone - Orphan - EMEA-002341-PIP02-23

Marinus Pharmaceuticals, Inc.; Treatment of tuberous sclerosis complex

Day 120 opinion

**Action**: For adoption

*Neurology*

### 2.1.7. Radiprodil - EMEA-003462-PIP01-23

Treatment of GRIN-related disorders

Day 120 opinion

**Action**: For adoption

*Neurology*

### 2.1.8. Recombinant adeno-associated virus Olig001 containing human aspartoacylase cDNA - Orphan - EMEA-003459-PIP01-23

Myrtelle, Inc.; Treatment of Canavan disease

Day 120 opinion

**Action**: For adoption
Neurology

2.1.9. **Brigimadlin - Orphan - EMEA-003260-PIP03-23**

Boehringer Ingelheim International GmbH; Treatment of soft tissue sarcoma excluding liposarcoma

Day 120 opinion

**Action**: For adoption

Oncology

2.1.10. **Humanised IgG1 monoclonal antibody against pituitary adenylate cyclase-activating polypeptide - EMEA-003483-PIP01-23**

Prevention of migraine

Day 120 opinion

**Action**: For adoption

Pain / Neurology

2.1.11. **Ezetimibe / rosuvastatin - EMEA-003582-PIP01-24**

Treatment of hypercholesterolaemia / Prevention of cardiovascular events

Day 60 opinion

**Action**: For adoption

Cardiovascular Diseases

2.1.12. **Gallium (68Ga) boclatixafortide - EMEA-003408-PIP02-24**

Diagnosis of primary aldosteronism

Day 60 opinion

**Action**: For adoption

Diagnostic

2.1.13. **Sitagliptin / dapagliflozin - EMEA-003572-PIP01-23**

Treatment of type 2 diabetes mellitus

Day 60 opinion

**Action**: For adoption

Endocrinology-Gynaecology-Fertility-Metabolism
### 2.1.14. 6-(4-((1s,3s)-1-amino-3-hydroxyclobutyl)phenyl)-1-ethyl-7-phenyl-1H-pyrido [2,3-b][1,4]oxazin-2(3H)-one, L-tartrate salt - Orphan - EMEA-003585-PIP01-24

Vaderis Therapeutics AG; Treatment of hereditary haemorrhagic telangiectasia

**Day 60 opinion**

**Action:** For adoption

**Haematology-Hemostaseology**

*Note: Withdrawal request received on 8 April 2024*

### 2.1.15. EMEA-003586-PIP01-24

Treatment of all solid and haematological malignancies

**Day 60 opinion**

**Action:** For adoption

**Oncology**

### 2.1.16. EMEA-003576-PIP01-24

Treatment of prostate cancer

**Day 60 opinion**

**Action:** For adoption

**Oncology**

### 2.1.17. Certepetide - Orphan - EMEA-003577-PIP01-24

Lisata Therapeutics Ireland Limited; Treatment of pancreatic cancer

**Day 60 opinion**

**Action:** For adoption

**Oncology**

### 2.1.18. EMEA-003581-PIP01-24

Treatment of lung cancer

**Day 60 opinion**

**Action:** For adoption

**Oncology**

### 2.1.19. Evencaleucel - Orphan - EMEA-003579-PIP01-24

XNK Therapeutics AB; Treatment of multiple myeloma
Day 60 opinion

**Action**: For adoption

Oncology

*Note: Withdrawal request received on 9 April 2024*

### 2.1.20. Rivoceranib - EMEA-002489-PIP02-24

Treatment of hepatocellular carcinoma

Day 60 opinion

**Action**: For adoption

Oncology

### 2.1.21. EMEA-003574-PIP01-24

Treatment of colorectal cancer

Day 60 opinion

**Action**: For adoption

Oncology

### 2.1.22. Lebrikizumab - EMEA-002536-PIP02-24

Treatment of chronic rhinosinusitis with nasal polyps

Day 60 opinion

**Action**: For adoption

Pneumology – Allergology

### 2.1.23. Fenofibrate / rosvastatin - EMEA-003591-PIP01-24

Treatment of mixed dyslipidemia

Day 30 opinion

**Action**: For adoption

Cardiovascular Diseases

### 2.1.24. Influenza virus surface antigens (haemagglutinin and neuraminidase) of strain B (Victoria lineage) / influenza virus surface antigens (haemagglutinin and neuraminidase) of strain A (H3N2) / influenza virus surface antigens (haemagglutinin and neuraminidase) of strain A (H1N1) - EMEA-003624-PIP01-24

Prevention of influenza infection

Day 30 opinion
**Action**: For adoption

Vaccines

### 2.2. Opinions on Compliance Check

#### 2.2.1. Maralixibat chloride - EMEA-C-001475-PIP03-17-M04

- Mirum Pharmaceuticals; Treatment of progressive familial intrahepatic cholestasis
- Day 60 opinion
- **Action**: For adoption
- Gastroenterology-Hepatology

#### 2.2.2. Itolizumab - EMEA-C1-003208-PIP02-22

- Biocon Pharma Malta I Limited; Treatment of acute graft versus host disease
- Day 60 letter
- **Action**: For adoption
- Immunology-Rheumatology-Transplantation

#### 2.2.3. Cobicistat / darunavir - EMEA-C4-001280-PIP01-12-M06

- Janssen-Cilag International NV; Treatment of human immunodeficiency virus (HIV-1) infection
- Day 60 letter
- **Action**: For adoption
- Infectious Diseases

#### 2.2.4. Binimetinib - EMEA-C-001454-PIP03-15-M03

- Pierre Fabre Médicament; Treatment of melanoma
- Day 60 opinion
- **Action**: For adoption
- Oncology

#### 2.2.5. Encorafenib - EMEA-C-001588-PIP01-13-M03

- Pierre Fabre Médicament; Treatment of melanoma
- Day 60 opinion
- **Action**: For adoption
Oncology

2.2.6. Pneumococcal polysaccharide conjugate vaccine (20-valent, adsorbed) - EMEA-C-002330-PIP01-18-M03

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

Day 60 opinion

**Action**: For adoption

Vaccines

2.2.7. Baloxavir marboxil - EMEA-C-002440-PIP01-18-M05

Roche Registration GmbH; Prevention of influenza

Day 30 opinion

**Action**: For adoption

Infectious Diseases

2.2.8. Canagliflozin - EMEA-C-001030-PIP01-10-M10

Janssen-Cilag International NV; Treatment of type 2 diabetes mellitus

Day 30 opinion

**Action**: For adoption

Endocrinology-Gynaecology-Fertility-Metabolism

2.2.9. Methoxyflurane - EMEA-C-000334-PIP01-08-M11

Medical Developments UK Ltd; Treatment of acute pain

Day 30 opinion

**Action**: For adoption

Pain

2.3. Opinions on Modification of an Agreed Paediatric Investigation Plan

2.3.1. Ertugliflozin - EMEA-001533-PIP01-13-M03

MSD Europe Belgium SRL; Treatment of type 2 diabetes mellitus

Day 60 opinion

**Action**: For adoption

Endocrinology-Gynaecology-Fertility-Metabolism
2.3.2. **Linaclotide - EMEA-000927-PIP01-10-M08**

AbbVie Deutschland GmbH & Co. KG; Treatment of functional constipation
Day 60 opinion
**Action:** For adoption
Gastroenterology-Hepatology

2.3.3. **Inebilizumab - EMEA-001911-PIP03-23-M01**

Horizon Therapeutics Ireland Designated Activity Company (DAC); Treatment of immunoglobulin G4-related disease (IgG4-RD)
Day 60 opinion
**Action:** For adoption
Immunology-Rheumatology-Transplantation

2.3.4. **Olokizumab - EMEA-001222-PIP01-11-M01**

Accelsiors GmbH; Treatment of chronic idiopathic arthritis (including rheumatoid arthritis, ankylosing spondylitis, psoriatic arthritis and juvenile idiopathic arthritis)
Day 60 opinion
**Action:** For adoption
Immunology-Rheumatology-Transplantation

2.3.5. **Relebactam monohydrate / cilastatin sodium / imipenem monohydrate - EMEA-001809-PIP01-15-M05**

MSD Europe Belgium SRL; Treatment of gram-negative bacterial infections
Day 60 opinion
**Action:** For adoption
Infectious Diseases

2.3.6. **Mecasermin rinfabate - Orphan - EMEA-000534-PIP03-17-M01**

OHB Neonatology Ltd; Prevention of chronic lung disease of prematurity
Day 60 opinion
**Action:** For adoption
Neonatology - Paediatric Intensive Care
2.3.7. Inebilizumab - EMEA-001911-PIP02-22-M01

Horizon Therapeutics Ireland Designated Activity Company (DAC); Treatment of myasthenia gravis
Day 60 opinion

**Action:** For adoption

Neurology

2.3.8. Soticlestat - Orphan - EMEA-002572-PIP02-19-M05

Takeda Pharma A/S; Treatment of Dravet syndrome / Treatment of Lennox-Gastaut syndrome
Day 60 opinion

**Action:** For adoption

Neurology

2.3.9. Tasimelteon - Orphan - EMEA-001531-PIP01-13-M05

Vanda Pharmaceuticals Netherlands B.V.; Treatment of non-24-hour sleep-wake disorder in the totally blind
Day 60 opinion

**Action:** For adoption

Neurology

*Note: Withdrawal request received on 16 April 2024*

2.3.10. Quizartinib - EMEA-001821-PIP01-15-M08

Daiichi Sankyo Europe GmbH; Treatment of acute myeloid leukaemia
Day 60 opinion

**Action:** For adoption

Oncology

2.3.11. Calcifediol - EMEA-002093-PIP02-17-M02

Vifor Fresenius Medical Care Renal Pharma France; Treatment of secondary hyperparathyroidism (SHPT)
Day 60 opinion

**Action:** For adoption

Uro-nephrology
2.3.12. Pegcetacoplan - Orphan - EMEA-002600-PIP03-21-M02

Apellis Ireland Limited; Treatment of glomerulonephritis and nephrotic syndrome

Day 60 opinion

**Action**: For adoption

Uro-nephrology

2.3.13. Neisseria meningitidis serogroup W polysaccharide conjugated to tetanus toxoid / Neisseria meningitidis serogroup Y polysaccharide conjugated to tetanus toxoid / Neisseria meningitidis serogroup C polysaccharide conjugated to tetanus toxoid / Neisseria meningitidis serogroup A polysaccharide conjugated to tetanus toxoid - EMEA-001930-PIP01-16-M05

Sanofi Pasteur; Prevention of invasive meningococcal disease

Day 60 opinion

**Action**: For adoption

Vaccines

2.3.14. mRNA encoding for the linked NTD and RBD domains of the spike glycoprotein of SARS-CoV-2 (mRNA-1283) - EMEA-003426-PIP01-23-M01


Day 60 opinion

**Action**: For adoption

Vaccines / Infectious Diseases

*Note: Withdrawal request received on 4 April 2024*

2.3.15. Obefazimod - EMEA-003196-PIP01-22-M01

Abivax; Treatment of ulcerative colitis

Day 30 opinion

**Action**: For adoption

Immunology-Rheumatology-Transplantation

2.4. Opinions on Re-examinations

2.4.1. Orforglipron - EMEA-003299-PIP01-22

Eli Lilly and Company; Treatment of type 2 diabetes mellitus

Day 30 opinion
**Action:** For adoption
Endocrinology-Gynaecology-Fertility-Metabolism

### 2.4.2. Sevasemten - EMEA-003394-PIP01-23

FGK Representative Service GmbH Germany; Treatment of dystrophinopathies

Day 30 opinion

**Action:** For adoption. Oral explanation to be held on Thursday 25 April 2024 at 14:00.

Other / Neurology

### 2.4.3. 13 Grass aqueous extract - EMEA-000813-PIP01-09-M01

Allergy Therapeutics (UK) Ltd.; Treatment of allergic rhinitis /rhino-conjunctivitis

Day 30 opinion

**Action:** For adoption

Pneumology – Allergology

### 2.4.4. Brexucabtagene autoleucel - Orphan - EMEA-001862-PIP03-20-M02

Kite Pharma EU B.V.; Treatment of mature B-cell neoplasms

Day 30 opinion

**Action:** For adoption

Oncology

### 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. Chikungunya virus virus-like particle vaccine / aluminum hydroxide - EMEA-C1-002656-PIP01-19-M01

Bavarian Nordic A/S; Prevention of chikungunya disease
### 2.7.2. Zuranolone - EMEA-C1-003119-PIP01-21-M01

Biogen Netherlands B.V.; Treatment of postpartum depression

Day 30 letter

**Action:** For information

**Vaccines / Infectious Diseases**

### 2.7.3. Ixazomib citrate - EMEA-C1-001410-PIP02-17-M04

Takeda Pharma A/S; Treatment of lymphoid malignancies (excluding multiple myeloma)

Day 30 letter

**Action:** For information

**Psychiatry**

### 2.7.4. Pridopidine (hydrochloride) - EMEA-C1-003174-PIP01-21-M01

Prilenia Therapeutics B.V.; Treatment of Huntington disease (HD)

Day 30 letter

**Action:** For information

**Neurology**

### 2.7.5. Chloroprocaine hydrochloride - EMEA-C2-000639-PIP03-16-M03

Sintetica GmbH; Peripheral nerve block (local anaesthesia by perineural injection)

Day 30 letter

**Action:** For information

**Anaesthesiology**

### 2.7.6. Fully human IgG1 RB-1 YTE anti-RSV F monoclonal antibody - EMEA-C1-002755-PIP01-19-M02

MSD Europe Belgium S.R.L.; Prevention of lower respiratory tract infection caused by respiratory syncytial virus

Day 30 letter

**Action:** For information
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. Ensitrelvir - EMEA-003192-PIP02-23

Treatment of coronavirus disease 2019 (COVID-19)

Day 90 discussion

Action: For discussion

Infectious Diseases

3.1.2. Lenacapavir - EMEA-002740-PIP02-23

Prevention of human immunodeficiency virus (HIV-1) infection

Day 90 discussion

Action: For discussion

Infectious Diseases

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

Recordati Rare Diseases; Treatment of neurotrophic keratitis

Day 90 discussion

Action: For discussion

Ophthalmology

3.1.4. Mirdametinib - Orphan - EMEA-003525-PIP01-23

Springworks Therapeutics Ireland Limited; Treatment of neurofibromatosis type 1 - plexiform neurofibroma / Treatment of neurofibromatosis type 1

Day 90 discussion

Action: For discussion

Other
3.1.5. EMEA-003451-PIP01-23

Treatment of bronchiectasis
Day 90 discussion
**Action:** For discussion
Pneumology - Allergology

3.1.6. EMEA-003580-PIP01-24

Treatment of elevated cholesterol / Treatment of mixed dyslipidaemia
Day 60 discussion
**Action:** For discussion
Cardiovascular Diseases

3.1.7. Ersodetug - Orphan - EMEA-002813-PIP02-24

Rezolute (Bio) Ireland Limited; Treatment of congenital hyperinsulinism
Day 60 discussion
**Action:** For discussion
Endocrinology-Gynaecology-Fertility-Metabolism

3.1.8. Crofelemer - Orphan - EMEA-003296-PIP02-24

Napo Therapeutics S.p.A.; Treatment of microvillus inclusion disease
Day 60 discussion
**Action:** For discussion
Gastroenterology-Hepatology

3.1.9. Mezagitamab - EMEA-003502-PIP02-24

Treatment of primary IgA nephropathy
Day 60 discussion
**Action:** For discussion
Haematology-Hemostaseology

3.1.10. Tildrakizumab - EMEA-001451-PIP02-24

Treatment of chronic idiopathic arthritis (including rheumatoid arthritis, psoriatic arthritis, ankylosing spondylitis and juvenile idiopathic arthritis)
### 3.1.11. Trofinetide - Orphan - EMEA-003587-PIP01-24

Acadia Pharmaceuticals Inc.; Treatment of Rett syndrome

**Action:** For discussion

**Immunology-Rheumatology-Transplantation**

### 3.1.12. 3,3-Dimethyl-N-(6-methyl-5-{[2-(1-methyl-1H-pyrazol-4-yl)pyridine-4-yl]oxy}pyridine-2-yl)-2-oxopyrrolidine-1-carboxamide hydrochloride hydrate - Orphan - EMEA-003495-PIP02-24

Abbisko Therapeutics., Co., Ltd.; Treatment of tenosynovial giant cell tumours

**Action:** For discussion

**Neurology**

### 3.1.13. 7-ethyl-10-hydroxy-camptothecin - Orphan - EMEA-003588-PIP01-24

CEBIOTEX S.L. Biomedical Nanofibers; Treatment of soft tissue neoplasms

**Action:** For discussion

**Oncology**


Treatment of melanoma

**Action:** For discussion

**Oncology**

### 3.1.15. Ravulizumab - EMEA-001943-PIP07-24

Treatment of primary IgA nephropathy

**Action:** For discussion
Uro-nephrology

3.1.16. mRNA encoding the influenza virus B/Victoria strain neuraminidase / mRNA encoding the influenza virus B/Victoria strain hemagglutinin / mRNA encoding the influenza virus H3N2 strain neuraminidase / mRNA encoding the influenza virus H3N2 strain hemagglutinin / mRNA encoding the influenza virus H1N1 strain neuraminidase / mRNA encoding the influenza virus H1N1 strain hemagglutinin - EMEA-003578-PIP01-24

Influenza immunisation
Day 60 discussion
**Action:** For discussion

Vaccines

3.1.17. Indapamide / ramipril - EMEA-003600-PIP01-24

Treatment of hypertension
Day 30 discussion
**Action:** For discussion

Cardiovascular Diseases

3.1.18. Allogeneic skin-derived ABCB5-positive dermal mesenchymal stromal cells - EMEA-002875-PIP02-24

Treatment of venous leg ulcer
Day 30 discussion
**Action:** For discussion

Dermatology

3.1.19. Autologous CD3-positive T cells transduced with a retroviral vector containing an anti-B cell maturation agent chimeric antigen receptor gene - EMEA-003593-PIP01-24

Treatment of systemic light chain amyloidosis
Day 30 discussion
**Action:** For discussion

Endocrinology-Gynaecology-Fertility-Metabolism

3.1.20. Teplizumab - EMEA-000524-PIP02-24

Prevention or delay of (clinical) type 1 diabetes mellitus
Day 30 discussion
3.1.21. **Interleukin-23 receptor antagonist peptide - EMEA-003301-PIP02-24**

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

Day 30 discussion

**Action**: For discussion

Endocrinology-Gynaecology-Fertility-Metabolism

3.1.22. **EMEA-003478-PIP02-24**

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

Day 30 discussion

**Action**: For discussion

Immunology-Rheumatology-Transplantation

3.1.23. **EMEA-003601-PIP01-24**

Prevention of *Clostridioides difficile* infection

Day 30 discussion

**Action**: For discussion

Infectious Diseases

3.1.24. **Bidridistrogene xeboparvovec - Orphan - EMEA-003400-PIP02-24**

Sarepta Therapeutics Ireland; Treatment of limb-girdle muscular dystrophy

Day 30 discussion

**Action**: For discussion

Neurology

3.1.25. **EMEA-003596-PIP01-24**

Treatment of mature B-cell neoplasms

Day 30 discussion

**Action**: For discussion

Oncology
3.1.26. **EMEA-003597-PIP01-24**

Treatment of pancreatic cancer
Day 30 discussion

**Action**: For discussion
Oncology

3.1.27. **EMEA-003598-PIP01-24**

Treatment of lung cancer
Day 30 discussion

**Action**: For discussion
Oncology

3.1.28. **Autologous CD3-positive T cells transduced with a retroviral vector containing an anti-B cell maturation agent chimeric antigen receptor gene - EMEA-003593-PIP02-24**

Treatment of multiple myeloma
Day 30 discussion

**Action**: For discussion
Oncology

3.1.29. **Fulzerasib - EMEA-003594-PIP01-24**

Treatment of colorectal cancer
Day 30 discussion

**Action**: For discussion
Oncology

3.1.30. **HER2 antibody drug conjugate - EMEA-003599-PIP01-24**

Treatment of breast cancer / Treatment of endometrial cancer
Day 30 discussion

**Action**: For discussion
Oncology

3.1.31. **Trastuzumab deruxtecan - EMEA-002978-PIP02-24**

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

Day 30 discussion

**Action:** For discussion

Oncology

### 3.1.32. 2′-O, 4′-C-methylene-P-thio-adenyl-(3′→5′)-2′-O, 4′-C-methylene-P-thio-guanayl-(3′→5′)-2′-O, 4′-C-methylene-P-thio-adenyl-(3′→5′)-2′-deoxy-P-thio-adenyl-(3′→5′)-2′-deoxy-P-thio-thymidylyl-(3′→5′)-2′-deoxy-P-thio-guanayl-(3′→5′)-2′-deoxy-P-thio-adenyl-(3′→5′)-2′-deoxy-P-thio-thymidylyl-(3′→5′)-2′-deoxy-P-thio-cytidylyl-(3′→5′)-2′-deoxy-P-thio-adenyl-(3′→5′)-2′-deoxy-P-thio-thymidylyl-(3′→5′)-2′-deoxy-P-thio-cytidylyl-(3′→5′)-2′-deoxy-P-thio-adenyl-(3′→5′)-2′-deoxy-P-thio-thymidylyl-(3′→5′)-2′-deoxy-P-thio-cytidylyl-(3′→5′)-2′-deoxy-P-thio-adenyl-(3′→5′)-2′-deoxy-P-thio-thymidylyl-(3′→5′)-2′-deoxy-P-thio-cytidylyl-(3′→5′)-2′-deoxy-P-thio-adenyl-(3′→5′)-2′-deoxy-P-thio-thymidylyl-(3′→5′)-2′-deoxy-P-thio-cytidylyl-(3′→5′)-2′-deoxy-P-thio-adenyl-(3′→5′)-2′-deoxy-P-thio-thymidylyl-(3′→5′)-2′-deoxy-P-thio-cytidylyl-(3′→5′)-2′-deoxy-P-thio-adenyl-(3′→5′)-2′-deoxy-P-thio-thymidylyl-(3′→5′)-2′-deoxy-P-thio-cytidylyl-(3′→5′)-2′-deoxy-P-thio-adenyl-

Ultragenyx Germany GmbH; Treatment of Angelman syndrome

Day 30 discussion

**Action:** For discussion

Other

### 3.1.33. Gorilla adenovirus vector expressing HPV6 and HPV11 antigens - Orphan - EMEA-003592-PIP01-24

Precigen, Inc.; Treatment of recurrent respiratory papillomatosis

Day 30 discussion

**Action:** For discussion

Oto-rhino-laryngology

### 3.1.34. Lebrikizumab - EMEA-002536-PIP03-24

Treatment of perennial allergic rhinitis

Day 30 discussion

**Action:** For discussion

Pneumology – Allergology

### 3.1.35. Influenza virus surface antigens (haemagglutinin and neuraminidase) of strain B (Victoria lineage) / influenza virus surface antigens (haemagglutinin and neuraminidase) of strain A (H3N2) / influenza virus surface antigens (haemagglutinin and neuraminidase) of strain A (H1N1) - EMEA-003623-PIP01-24

Prophylaxis of influenza

Day 30 discussion
**Action**: For discussion

### 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. Olipudase alfa - EMEA-C-001600-PIP01-13-M02

Sanofi B.V.; Treatment of Niemann-Pick disease

Day 30 discussion

**Action**: For discussion

Endocrinology - Gynaecology - Fertility - Metabolism

#### 3.2.2. Tedizolid phosphate - EMEA-C3-001379-PIP01-12-M08

MSD Europe Belgium SRL; Treatment of acute bacterial skin and skin structure infections

Day 30 discussion

**Action**: For discussion

Infectious Diseases

#### 3.2.3. Vanzacaftor / tezacaftor / deutivacaftor - EMEA-C1-003052-PIP01-21

Vertex Pharmaceuticals (Ireland) Limited; Treatment of cystic fibrosis

Day 30 discussion

**Action**: For discussion

Pneumology - Allergology

#### 3.2.4. Sepiapterin – Orphan - EMEA-C1-003027-PIP02-23

PTC Therapeutics International Limited; Treatment of hyperphenylalaninemia

Day 30 discussion

**Action**: For discussion

Endocrinology - Gynaecology - Fertility - Metabolism

#### 3.2.5. Fordadistrogene movaparvovec - EMEA-C1-002741-PIP01-20-M02

Pfizer Europe MA EEIG; Treatment of Duchenne muscular dystrophy

Day 30 discussion
**Action**: For discussion

Neurology

### 3.2.6. Sargramostim - EMEA-C1-003568-PIP01-23

Sargramostim Partner Therapeutics; Treatment of patients acutely exposed to myelosuppressive doses of radiation (hematopoietic syndrome of acute radiation syndrome – H-ARS)

Day 30 discussion

**Action**: For discussion

Immunology – Rheumatology – Transplantation

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

#### 3.3.1. Ralinepag - Orphan - EMEA-002432-PIP02-20-M01

United Therapeutics Corporation; Treatment of pulmonary arterial hypertension

Day 30 discussion

**Action**: For discussion

Cardiovascular Diseases

#### 3.3.2. Regadenoson - EMEA-000410-PIP01-08-M07

GE Healthcare AS; Diagnosis of myocardial perfusion disturbances

Day 30 discussion

**Action**: For discussion

Diagnostic / Cardiovascular Diseases

#### 3.3.3. Pegvaliase - Orphan - EMEA-001951-PIP01-16-M03

BioMarin International Limited; Treatment of hyperphenylalaninaemia

Day 30 discussion

**Action**: For discussion

Endocrinology-Gynaecology-Fertility-Metabolism

#### 3.3.4. RAAV8-hUGT1A1 - Orphan - EMEA-002021-PIP01-16-M01

GENETHON; Treatment of Crigler-Najjar syndrome

Day 30 discussion
| **Action**: For discussion |  |
| Endocrinology-Gynaecology-Fertility-Metabolism |  |

### 3.3.5. Vedolizumab - EMEA-000645-PIP01-09-M09

Takeda Pharma A/S; Treatment of ulcerative colitis / Treatment of Crohn's disease  
Day 30 discussion  
**Action**: For discussion  
Gastroenterology-Hepatology

### 3.3.6. Luspatercept - Orphan - EMEA-001521-PIP01-13-M07

Bristol-Myers Squibb Pharma EEIG; Treatment of myelodysplastic syndromes / Treatment of beta-thalassaemia  
Day 30 discussion  
**Action**: For discussion  
Haematology-Hemostaseology

### 3.3.7. Vonicog alfa - EMEA-001164-PIP01-11-M08

Baxalta Innovations GmBH; Treatment of Von Willebrand disease  
Day 30 discussion  
**Action**: For discussion  
Haematology-Hemostaseology

### 3.3.8. Sarilumab - EMEA-001045-PIP01-10-M04

Sanofi Winthrop Industrie; Treatment of chronic idiopathic arthritis (including rheumatoid arthritis, spondylarthritis, psoriatic arthritis and juvenile idiopathic arthritis)  
Day 30 discussion  
**Action**: For discussion  
Immunology-Rheumatology-Transplantation

### 3.3.9. Tofacitinib citrate - EMEA-000576-PIP01-09-M16

Pfizer Europe MA EEIG; Treatment of chronic idiopathic arthritis (including rheumatoid arthritis, psoriatic arthritis, ankylosing spondylitis and juvenile idiopathic arthritis)  
Day 30 discussion  
**Action**: For discussion  
Immunology-Rheumatology-Transplantation
3.3.10. Islatravir / doravirine - EMEA-002707-PIP01-19-M02

MSD Europe Belgium SRL; Treatment of human immunodeficiency virus-1 (HIV-1) infection

Day 30 discussion

Action: For discussion

Infectious Diseases

3.3.11. Lamivudine / dolutegravir - EMEA-001940-PIP01-16-M06

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

Day 30 discussion

Action: For discussion

Infectious Diseases

3.3.12. Tenofovir disoproxil fumarate / lamivudine / doravirine - EMEA-001695-PIP01-14-M06

MSD Europe Belgium SRL; Treatment of human immunodeficiency virus-1 (HIV-1) infection

Day 30 discussion

Action: For discussion

Infectious Diseases

3.3.13. Ublituximab - EMEA-002889-PIP02-20-M01

Neuraxpharm Pharmaceuticals, S.L.; Treatment of multiple sclerosis

Day 30 discussion

Action: For discussion

Neurology


Kite Pharma EU B.V.; Treatment of acute lymphoblastic leukaemia

Day 30 discussion

Action: For discussion

Oncology

3.3.15. Dostarlimab - EMEA-002463-PIP01-18-M02

GlaxoSmithKline (Ireland) Limited; Treatment of all conditions included in the category of malignant neoplasms (except haematopoietic and lymphoid malignancies)

Day 30 discussion
3.3.16. **Epcoritamab - Orphan - EMEA-002907-PIP01-20-M03**

Abbvie Ltd; Treatment of mature B-cell lymphoma  
Day 30 discussion  
**Action**: For discussion  
Oncology

3.3.17. **Niraparib tosylate monohydrate - EMEA-002268-PIP02-18-M02**

GlaxoSmithKline (Ireland) Limited; Treatment of all conditions included in the category of malignant neoplasms (except haematopoietic and lymphoid malignancies)  
Day 30 discussion  
**Action**: For discussion  
Oncology

3.3.18. **Ribociclib - EMEA-002765-PIP02-21-M01**

Novartis Europharm Limited; Treatment of neuroblastoma  
Day 30 discussion  
**Action**: For discussion  
Oncology

3.3.19. **Botaretigene sparoparvovec - Orphan - EMEA-002827-PIP01-20-M03**

Janssen-Cilag International NV Turnhoutseweg 30; Treatment of retinitis pigmentosa  
Day 30 discussion  
**Action**: For discussion  
Ophthalmology

3.3.20. **Iptacopan - Orphan - EMEA-002705-PIP01-19-M02**

Novartis Europharm Limited; Treatment of C3 glomerulopathy  
Day 30 discussion  
**Action**: For discussion  
Other
3.3.21. Mexiletine hydrochloride - Orphan - EMEA-002012-PIP01-16-M05

Lupin Europe GmbH; Treatment of myotonic disorders
Day 30 discussion
*Action*: For discussion
Other

3.3.22. Setrusumab - Orphan - EMEA-002169-PIP01-17-M03

Mereo BioPharma Ireland Limited; Treatment of osteogenesis imperfecta
Day 30 discussion
*Action*: For discussion
Other

3.3.23. Xylitol / procaine hydrochloride / magnesium sulphate heptahydrate / potassium chloride - EMEA-001171-PIP01-11-M03

MIT Gesundheit GmbH; Cardioplegia
Day 30 discussion
*Action*: For discussion
Other

3.3.24. Dermatophagoides farinae extracts - EMEA-000834-PIP01-10-M01

Allergopharma GmbH & Co. KG; Treatment of allergic rhinitis / rhinoconjunctivitis
Day 30 discussion
*Action*: For discussion
Pneumology - Allergology

3.3.25. Dermatophagoides pteronyssinus extracts 100% - EMEA-000835-PIP01-10-M01

Allergopharma GmbH & Co. KG; Treatment of allergic rhinitis / rhinoconjunctivitis
Day 30 discussion
*Action*: For discussion
Pneumology - Allergology

3.3.26. Dermatophagoides pteronyssinus / Dermatophagoides farinae extracts (50%/50%) - EMEA-000836-PIP01-10-M01

Allergopharma GmbH & Co. KG; Treatment of allergic rhinitis / rhinoconjunctivitis
Day 30 discussion
3.3.27. Cariprazine hydrochloride - EMEA-001652-PIP01-14-M06

Gedeon Richter Plc.; Treatment of schizophrenia

Day 30 discussion

Action: For discussion

Psychiatry

3.3.28. Ravulizumab (ALXN1210) - EMEA-001943-PIP02-20-M03

Alexion Europe SAS; Treatment in haematopoietic stem cell transplant

Day 30 discussion

Action: For discussion

Uro-nephrology / Haematology-Hemostaseology

3.3.29. NVX-CoV2373 - EMEA-002941-PIP01-20-M05

Novavax CZ, a.s.; Prevention of coronavirus disease 2019 (COVID-19)

Day 30 discussion

Action: For discussion

Vaccines

3.3.30. COVID-19 Vaccine, recombinant, adjuvanted - EMEA-003191-PIP01-22-M01

HIPRA Human Health S.L.U.; Prevention of coronavirus disease 2019 (COVID-19)

Day 30 discussion

Action: For discussion

Vaccines

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 29 April 2024 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**

6.1.1. Cisplatin - EMEA-03-2024

InhaTarget Therapeutics SRL; The class of first- and second generation platinum-containing medicinal products for treatment of lung malignant neoplasms; Treatment of non-small cell lung cancer (NSCLC)

**Action:** For adoption

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

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

7.1.1. Zibotentan / dapagliflozin (propanediol monohydrate) - EMEA-002969-PIP01-21

AstraZeneca AB; Treatment of chronic kidney disease

Proposed indication: Treatment of adults with IgA nephropathy

**Action:** For adoption
7.1.2. Autologous CD3+CD4+CD25+CD127-FoxP3+ polyclonal regulatory T cells ex vivo expanded - EMEA-002737-PIP01-19

PolTreg SA; Treatment of type 1 diabetes mellitus

Proposed indication: Treatment of presymptomatic (stage 1) diabetes mellitus type 1 (T1DM stage 1)

Action: For adoption

8. Annual reports on deferrals

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

9. Organisational, regulatory and methodological matters

9.1. Mandate and organisation of the PDCO

9.1.1. PDCO membership

Action: For information

9.1.2. Vote by Proxy

Action: For information

9.1.3. Strategic Review and Learning Meeting (SRLM) - Leuven, Belgium, 16-17 May 2024

Update on the SRLM meeting to be held in Leuven during the Belgium Presidency

PDCO member: Karen van Malderen

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

9.2.2. Committee on Herbal Medicinal Products (HMPC) - Reflection paper on data recommendations for (traditional) herbal medicinal products in children/adolescents

PDCO member: Peter Sisovsky

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. **Paediatric Formulation Operational Expert Group (PFOEG)**

PDCO member: Brian Aylward (ad interim)

**Action**: For information


No item

9.3.4. **Upcoming Innovation Task Force (ITF) meetings**

**Action**: For information

9.4. **Cooperation within the EU regulatory network**

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

No item

9.5. **Cooperation with International Regulators**

9.5.1. **Paediatric Cluster Teleconference**

**Action**: For information

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

No item

9.7. **PDCO work plan**

No item
9.8.  Planning and reporting

9.8.1.  EMA Business Pipeline activity and Horizon scanning

No item

9.8.2.  Marketing Authorisation Applications (MAAs) 3-year forecast report (March 2024 to December 2026)

Action: For information

10.  Any other business

10.1.  Accelerating Clinical Trials (ACT) EU PA7 pilot between Scientific Advice Working Party (SAWP) and Clinical Trials Coordination Group (CTCG)

Action: For information

10.2.  Onboarding of Paediatric processes on IRIS

Scope: The PDCO will be informed about the progress of onboarding paediatric procedures in IRIS and will be shown a brief live demo

Action: For information

10.3.  Update on the Accelerating Clinical Trials (ACT) EU PA8 workshop

Action: For information

10.4.  Feedback on training on PIPs for the Clinical Trials Coordination Group (CTCG) assessor round table

PDCO member: Anette Solli Karlsen

Action: For information

11.  Breakout sessions

11.1.  Internal PDCO Operations

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

11.2.  Neonatology

Action: For discussion on Wednesday, 13:00 - 14:00
11.3. **HIV**

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

11.4. **Paediatric oncology**

**Action**: For discussion on Thursday, 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.

For a list of acronyms and abbreviations, see: 
Abbreviations used in EMA scientific committees & CMD documents and in relation to EMA’s regulatory activities

More detailed information on the above terms can be found on the EMA website: www.ema.europa.eu/