



EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

18 April 2024
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Human Medicines Division

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

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 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. Zascotinib - 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-hydroxycyclobutyl\)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 / rosuvastatin - 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

MODERNA BIOTECH SPAIN, S.L.; Prevention of coronavirus disease 2019 (COVID-19)

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

Day 30 letter

Action: For information

Vaccines / Infectious Diseases

2.7.2. Zuranolone - EMEA-C1-003119-PIP01-21-M01

Biogen Netherlands B.V.; Treatment of postpartum depression

Day 30 letter

Action: For information

Psychiatry

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

Oncology

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. Chlorprocaine 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)

Day 60 discussion

Action: For discussion

Immunology-Rheumatology-Transplantation

3.1.11. Trofinetide - Orphan - EMEA-003587-PIP01-24

Acadia Pharmaceuticals Inc.; Treatment of Rett syndrome

Day 60 discussion

Action: For discussion

Neurology

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

Day 60 discussion

Action: For discussion

Oncology

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

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

Day 60 discussion

Action: For discussion

Oncology

3.1.14. Autologous T-cells from a melanoma metastasis - EMEA-003535-PIP02-24

Treatment of melanoma

Day 60 discussion

Action: For discussion

Oncology

3.1.15. Ravulizumab - EMEA-001943-PIP07-24

Treatment of primary IgA nephropathy

Day 60 discussion

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

Action: For discussion

Endocrinology-Gynaecology-Fertility-Metabolism

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

Immunology-Rheumatology-Transplantation

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-adenylyl-(3'→5')-2'-O, 4'-C-methylene-P-thio-guanylyl-(3'→5')-2'-O, 4'-C-methylene-P-thio-adenylyl-(3'→5')-2'-deoxy-P-thio-adenylyl-(3'→5')-2'-deoxy-P-thio-thymidylyl-(3'→5')-2'-deoxy-P-thio-guanylyl-(3'→5')-2'-deoxy-P-thio-guanylyl-(3'→5')-2'-deoxy-P-thio-cytidylyl-(3'→5')-2'-deoxy-P-thio-adenylyl-(3'→5')-2'-deoxy-P-thio-cytidylyl-(3'→5')-2'-deoxy-P-thio-adenylyl-(3'→5')-2'-deoxy-P-thio-thymidylyl-(3'→5')- 2'-deoxy-P-thio-cytidylyl-(3'→5')-2'-deoxy-P-thio-thymidylyl-(3'→5')-2'-O, 4'-C-methylene-5-methyl-P-thio-cytidylyl-(3'→5')-2'-O, 4'-C-methylene-5-methyl-P-thio-uridylyl-(3'→5')-2'-O, 4'-C-methylene-5-methyl-P-thio-uridylyl-(3'→5')-2'-O, 4'-C-methyleneguanosine, heptadecasodium salt - Orphan - EMEA-003595-PIP01-24
-

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

Vaccines

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

3.3.14. Brexucabtagene autoleucel - Orphan - EMEA-001862-PIP01-15-M04

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

Action: For discussion

Oncology

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 sparaparvovec - 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

Action: For discussion

Pneumology - Allergology

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

9.3.3. Patients and Consumers Working Party (PCWP)/Healthcare Professionals Working Party (HCPWP)

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/