



EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

24 May 2024
EMA/PDCO/189594/2024
Human Medicines Division

Paediatric Committee (PDCO)

Draft Agenda for the meeting on 28-31 May 2024

Chair: Brian Aylward – Vice-Chair: Sylvie Benchetrit

28 May 2024, 14:00 - 19:00, Virtual meeting

29 May 2024, 08:30 - 19:00, Virtual meeting

30 May 2024, 08:30 - 19:00, Virtual meeting

31 May 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 ongoing 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 28-31 May 2024. See May 2024 PDCO minutes (to be published post June 2024 PDCO meeting).

1.2. Adoption of agenda

PDCO agenda for 28-31 May 2024.

1.3. Adoption of the minutes

PDCO minutes for 23-26 April 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. Ensitrelvir - EMEA-003192-PIP02-23

Treatment of coronavirus disease 2019 (COVID-19)

Day 120 opinion

Action: For adoption

Infectious Diseases

2.1.2. Lenacapavir - EMEA-002740-PIP02-23

Prevention of human immunodeficiency virus (HIV-1) infection

Day 120 opinion

Action: For adoption

Infectious Diseases

Note: Withdrawal request received on 21 May 2024

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

Recordati Rare Diseases; Treatment of neurotrophic keratitis

Day 120 opinion

Action: For adoption

Ophthalmology

Note: Withdrawal request received on 21 May 2024

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

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

Day 120 opinion

Action: For adoption

Other

2.1.5. Derivative of azabicycloheptane-carboxamide - EMEA-003451-PIP01-23

Treatment of bronchiectasis

Day 120 opinion

Action: For adoption

Pneumology - Allergology

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

Treatment of hypertension

Day 60 opinion

Action: For adoption

Cardiovascular Diseases

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

Treatment of venous leg ulcer

Day 60 opinion

Action: For adoption

Dermatology

2.1.8. 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 60 opinion

Action: For adoption

Endocrinology-Gynaecology-Fertility-Metabolism

2.1.9. EMEA-003596-PIP01-24

Treatment of mature B-cell neoplasms

Day 60 opinion

Action: For adoption

Oncology

2.1.10. EMEA-003597-PIP01-24

Treatment of pancreatic cancer

Day 60 opinion

Action: For adoption

Oncology

2.1.11. Anti-human LAG-3 mAb, human IgG4 isotype - EMEA-003598-PIP01-24

Treatment of lung cancer

Day 60 opinion

Action: For adoption

Oncology

2.1.12. 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 60 opinion

Action: For adoption

Oncology

2.1.13. Fulzerasib - EMEA-003594-PIP01-24

Treatment of colorectal cancer

Day 60 opinion

Action: For adoption

Oncology

2.1.14. HER2 antibody drug conjugate - EMEA-003599-PIP01-24

Treatment of breast cancer / Treatment of endometrial cancer

Day 60 opinion

Action: For adoption

Oncology

2.1.15. Recombinant influenza hemagglutinin-strain A (H1N1 subtype) / recombinant influenza hemagglutinin-strain A (H3N2 subtype) / recombinant influenza hemagglutinin-strain B - EMEA-003640-PIP01-24

Prevention of influenza disease

Day 30 opinion

Action: For adoption

Vaccines

2.1.16. Diclofenac / thicolchicoside - EMEA-003611-PIP01-24

Treatment of musculoskeletal and connective tissue pain and discomfort

Day 30 opinion

Action: For adoption

Pain

2.2. Opinions on Compliance Check

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

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

Day 60 opinion

Action: For adoption

Endocrinology-Gynaecology-Fertility-Metabolism

2.2.2. Tedizolid phosphate - EMEA-C3-001379-PIP01-12-M08

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

Day 60 letter

Action: For adoption

Infectious Diseases

2.2.3. Vanzacaftor / tezacaftor / deutivacaftor - EMEA-C1-003052-PIP01-21

Vertex Pharmaceuticals (Ireland) Limited; Treatment of cystic fibrosis

Day 60 letter

Action: For adoption

Pneumology – Allergology

2.2.4. Landiolol (hydrochloride) - EMEA-C-001150-PIP02-13-M05

AOP Orphan Pharmaceuticals GmbH; Treatment of supraventricular arrhythmias

Day 30 opinion

Action: For adoption

Cardiovascular Diseases

2.2.5. Fordadistrogene movaparvovec - EMEA-C1-002741-PIP01-20-M02

Pfizer Europe MA EEIG; Treatment of Duchenne muscular dystrophy

Day 60 letter

Action: For adoption

Neurology

2.3. Opinions on Modification of an Agreed Paediatric Investigation Plan

2.3.1. Ralinepag - Orphan - EMEA-002432-PIP02-20-M01

United Therapeutics Corporation; Treatment of pulmonary arterial hypertension

Day 60 opinion

Action: For adoption

Cardiovascular Diseases

[2.3.2. Regadenoson - EMEA-000410-PIP01-08-M07](#)

GE Healthcare AS; Diagnosis of myocardial perfusion disturbances

Day 60 opinion

Action: For adoption

Diagnostic / Cardiovascular Diseases

[2.3.3. Pegvaliase - Orphan - EMEA-001951-PIP01-16-M03](#)

BioMarin International Limited; Treatment of hyperphenylalaninaemia

Day 60 opinion

Action: For adoption

Endocrinology-Gynaecology-Fertility-Metabolism

[2.3.4. RAAV8-hUGT1A1 - Orphan - EMEA-002021-PIP01-16-M01](#)

GENETHON; Treatment of Crigler-Najjar syndrome

Day 60 opinion

Action: For adoption

Endocrinology-Gynaecology-Fertility-Metabolism

[2.3.5. Vedolizumab - EMEA-000645-PIP01-09-M09](#)

Takeda Pharma A/S; Treatment of ulcerative colitis / Treatment of Crohn's disease

Day 60 opinion

Action: For adoption

Gastroenterology-Hepatology

[2.3.6. Luspatercept - Orphan - EMEA-001521-PIP01-13-M07](#)

Bristol-Myers Squibb Pharma EEIG; Treatment of myelodysplastic syndromes / Treatment of beta-thalassaemia

Day 60 opinion

Action: For adoption

Haematology-Hemostaseology

[2.3.7. Vonicog alfa - EMEA-001164-PIP01-11-M08](#)

Baxalta Innovations GmbH; Treatment of Von Willebrand disease

Day 60 opinion

Action: For adoption

Haematology-Hemostaseology

2.3.8. 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 60 opinion

Action: For adoption

Immunology-Rheumatology-Transplantation

2.3.9. 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 60 opinion

Action: For adoption

Immunology-Rheumatology-Transplantation

2.3.10. Islatravir / doravirine - EMEA-002707-PIP01-19-M02

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

Day 60 opinion

Action: For adoption

Infectious Diseases

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

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

Day 60 opinion

Action: For adoption

Infectious Diseases

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

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

Day 60 opinion

Action: For adoption

Infectious Diseases

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

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

Day 60 opinion

Action: For adoption

Neurology

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

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

Day 60 opinion

Action: For adoption

Oncology

2.3.15. 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 60 opinion

Action: For adoption

Oncology

2.3.16. Epcoritamab - Orphan - EMEA-002907-PIP01-20-M03

AbbVie Ltd; Treatment of mature B-cell lymphoma

Day 60 opinion

Action: For adoption

Oncology

2.3.17. 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 60 opinion

Action: For adoption

Oncology

2.3.18. Ribociclib - EMEA-002765-PIP02-21-M01

Novartis Europharm Limited; Treatment of neuroblastoma

Day 60 opinion

Action: For adoption

Oncology

2.3.19. Botaretigene sparoparvovec - Orphan - EMEA-002827-PIP01-20-M03

Janssen-Cilag International NV Turnhoutseweg 30; Treatment of retinitis pigmentosa

Day 60 opinion

Action: For adoption

Ophthalmology

2.3.20. Iptacopan - Orphan - EMEA-002705-PIP01-19-M02

Novartis Europharm Limited; Treatment of C3 glomerulopathy

Day 60 opinion

Action: For adoption

Other

2.3.21. Mexiletine hydrochloride - Orphan - EMEA-002012-PIP01-16-M05

Lupin Europe GmbH; Treatment of myotonic disorders

Day 60 opinion

Action: For adoption

Other

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

Mereo BioPharma Ireland Limited; Treatment of osteogenesis imperfecta

Day 60 opinion

Action: For adoption

Other

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

MIT Gesundheit GmbH; Cardioplegia

Day 60 opinion

Action: For adoption

Other

2.3.24. *Dermatophagoides farinae* extracts - EMEA-000834-PIP01-10-M01

Allergopharma GmbH & Co. KG; Treatment of allergic asthma / Treatment of allergic rhinitis / rhinoconjunctivitis

Day 60 opinion

Action: For adoption

Pneumology - Allergology

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

Allergopharma GmbH & Co. KG; Treatment of allergic asthma / Treatment of allergic rhinitis / rhinoconjunctivitis

Day 60 opinion

Action: For adoption

Pneumology - Allergology

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

Allergopharma GmbH & Co. KG; Treatment of allergic rhinitis / rhinoconjunctivitis / Treatment of allergic asthma

Day 60 opinion

Action: For adoption

Pneumology - Allergology

2.3.27. Cariprazine hydrochloride - EMEA-001652-PIP01-14-M06

Gedeon Richter Plc.; Treatment of schizophrenia

Day 60 opinion

Action: For adoption

Psychiatry

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

Alexion Europe SAS; Treatment in haematopoietic stem cell transplant

Day 60 opinion

Action: For adoption

Uro-nephrology / Haematology-Hemostaseology

Note: Withdrawal request received on 14 May 2024

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

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

Day 60 opinion

Action: For adoption

Vaccines

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

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

Day 60 opinion

Action: For adoption

Vaccines

2.3.31. Recombinant influenza hemagglutinin-strain B (Yamagata lineage) / recombinant influenza hemagglutinin-strain B (Victoria lineage) / recombinant influenza hemagglutinin-strain A (H3N2 subtype) / recombinant influenza hemagglutinin-strain A (H1N1 subtype) - EMEA-002418-PIP01-18-M04

Sanofi Pasteur; Prevention of influenza disease

Day 30 opinion

Action: For adoption

Vaccines

2.3.32. Ibrexafungerp (citrate) - EMEA-002535-PIP03-19-M01

SCYNEXIS, Inc.; Treatment of vulvovaginal candidiasis / Prevention of recurrent vulvovaginal candidiasis

Day 30 opinion

Action: For adoption

Infectious Diseases

2.3.33. Gefurulumab - EMEA-003302-PIP01-22-M01

Alexion Europe SAS; Treatment of myasthenia gravis

Day 30 opinion

Action: For adoption

Neurology

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. Onasemnogene abeparvovec - EMEA-C2-002168-PIP01-17-M05

Novartis Europharm Limited; Treatment of spinal muscular atrophy

Day 30 letter

Action: For information

Neurology

2.7.2. Gadoquatrane - EMEA-C1-002778-PIP01-20-M01

Bayer AG; Diagnosis by evaluation of any known or suspected clinical condition with contrast enhanced magnetic resonance imaging

Day 30 letter

Action: For information

Diagnostic

2.7.3. Ustekinumab - EMEA-C2-000311-PIP04-13-M06

Janssen-Cilag International NV; Treatment of Crohn's disease

Day 30 letter

Action: For information

Gastroenterology-Hepatology

2.7.4. Acetyl-L-leucine - EMEA-C1-002796-PIP01-20-M02

IntraBio Ltd; Treatment of Niemann-Pick disease type C

Day 30 letter

Action: For information

Neurology

2.7.5. Iptacopan - EMEA-C1-002705-PIP02-19-M01

Novartis Europharm Limited; Treatment of IgA nephropathy

Day 30 letter

Action: For information

Uro-nephrology

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. EMEA-003513-PIP01-23

Treatment of coeliac disease

Day 90 discussion

Action: For discussion

Gastroenterology-Hepatology

3.1.2. Mezagitamab - EMEA-003502-PIP01-23

Treatment of immune thrombocytopenia (ITP)

Day 90 discussion

Action: For discussion

Haematology-Hemostaseology

3.1.3. Ganaxolone - EMEA-002341-PIP03-23

Treatment of refractory status epilepticus

Day 90 discussion

Action: For discussion

Neurology

3.1.4. Vosoritide - EMEA-002033-PIP02-23

Treatment of hypochondroplasia

Day 90 discussion

Action: For discussion

Other

3.1.5. A/H1N1, A/H3N2, and B/Victoria and single-stranded 5' capped mRNA encoding the HAs of the influenza virus strains A/H1N1, A/H3N2, and B/Victoria and the N-terminal domain (NTD) and receptor binding domain (RBD) of the SARS-CoV-2 spike glycoprotein - EMEA-003521-PIP01-23

Prevention of influenza and coronavirus disease 2019 (COVID-19)

Day 90 discussion

Action: For discussion

Vaccines

3.1.6. Teplizumab - EMEA-000524-PIP02-24

Prevention or delay of (clinical) type 1 diabetes mellitus

Day 60 discussion

Action: For discussion

Endocrinology-Gynaecology-Fertility-Metabolism

3.1.7. 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 60 discussion

Action: For discussion

Immunology-Rheumatology-Transplantation

3.1.8. EMEA-003478-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

3.1.9. EMEA-003601-PIP01-24

Prevention of *Clostridioides difficile* infection

Day 60 discussion

Action: For discussion

Infectious Diseases

3.1.10. Bidridistrogene xeboparvovec - Orphan - EMEA-003400-PIP02-24

Sarepta Therapeutics Ireland; Treatment of limb-girdle muscular dystrophy

Day 60 discussion

Action: For discussion

Neurology

3.1.11. Trastuzumab deruxtecan - EMEA-002978-PIP02-24

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

Day 60 discussion

Action: For discussion

Oncology

3.1.12. 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 60 discussion

Action: For discussion

Other

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

Precigen, Inc.; Treatment respiratory papillomatosis

Day 60 discussion

Action: For discussion

Oto-rhino-laryngology

3.1.14. Lebrikizumab - EMEA-002536-PIP03-24

Treatment of perennial allergic rhinitis

Day 60 discussion

Action: For discussion

Pneumology - Allergology

3.1.15. Tadalafil / ambrisentan - EMEA-003617-PIP01-24

Treatment of pulmonary arterial hypertension

Day 30 discussion

Action: For discussion

Cardiovascular Diseases

3.1.16. Tadalafil / ambrisentan - EMEA-003621-PIP01-24

Treatment of pulmonary arterial hypertension

Day 30 discussion

Action: For discussion

Cardiovascular Diseases

3.1.17. Luliconazole - EMEA-003604-PIP01-24

Treatment of onychomycosis

Day 30 discussion

Action: For discussion

Dermatology

3.1.18. Tarumase - EMEA-003616-PIP01-24

Treatment of chronic wounds

Day 30 discussion

Action: For discussion

Dermatology

3.1.19. [Copper \(⁶⁴Cu\) oxodotreotide - Orphan - EMEA-003610-PIP01-24](#)

Curium Pet France; Diagnosis of neuroendocrine neoplasms

Day 30 discussion

Action: For discussion

Diagnostic

3.1.20. [2-isopropyl-3H-naphtho\[1,2-d\]imidazole-4,5-dione - Orphan - EMEA-003618-PIP01-24](#)

Abliva AB; Treatment of primary mitochondrial disease

Day 30 discussion

Action: For discussion

Endocrinology-Gynaecology-Fertility-Metabolism

3.1.21. [Alvelestat - EMEA-003605-PIP01-24](#)

Treatment of alpha-1 antitrypsin deficiency

Day 30 discussion

Action: For discussion

Endocrinology-Gynaecology-Fertility-Metabolism

3.1.22. [Synthetic double-stranded siRNA oligonucleotide directed against apolipoprotein C-III mRNA and covalently linked to a ligand containing three N-acetylgalactosamine residues - EMEA-003420-PIP02-24](#)

Treatment of severe hypertriglyceridaemia (SHTG)

Day 30 discussion

Action: For discussion

Endocrinology-Gynaecology-Fertility-Metabolism

3.1.23. [Diazoxide choline - Orphan - EMEA-003614-PIP01-24](#)

Soleno Therapeutics Europe Ltd.; Treatment of Prader-Willi syndrome

Day 30 discussion

Action: For discussion

Endocrinology-Gynaecology-Fertility-Metabolism / Psychiatry / Neurology

3.1.24. [Pegozafermin - EMEA-003619-PIP01-24](#)

Treatment of metabolic dysfunction-associated steatohepatitis (MASH)

Day 30 discussion

Action: For discussion

Gastroenterology-Hepatology

3.1.25. [Inobrodib - EMEA-003622-PIP01-24](#)

Treatment of multiple myeloma

Day 30 discussion

Action: For discussion

Haematology-Hemostaseology

3.1.26. [Telitacicept - EMEA-002824-PIP02-24](#)

Treatment of myasthenia gravis

Day 30 discussion

Action: For discussion

Immunology-Rheumatology-Transplantation

3.1.27. [Autologous CD34+ haematopoietic stem cells transduced ex vivo with a lentiviral vector encoding human Wiskott-Aldrich syndrome protein - EMEA-003620-PIP01-24](#)

Treatment of Wiskott-Aldrich syndrome

Day 30 discussion

Action: For discussion

Immunology-Rheumatology-Transplantation / Haematology-Hemostaseology

3.1.28. [EMEA-003609-PIP01-24](#)

Treatment of respiratory tract disease caused by human respiratory syncytial virus

Day 30 discussion

Action: For discussion

Infectious Diseases

3.1.29. [EMEA-003306-PIP02-24](#)

Treatment of human respiratory syncytial virus (RSV)

Day 30 discussion

Action: For discussion

Infectious Diseases

3.1.30. [EMEA-003615-PIP01-24](#)

Treatment of Prader Willi syndrome

Day 30 discussion

Action: For discussion

Neurology

3.1.31. [Frexalimab - EMEA-002945-PIP04-24](#)

Treatment of multiple sclerosis

Day 30 discussion

Action: For discussion

Neurology

3.1.32. [Acasunlimab - EMEA-003606-PIP01-24](#)

Treatment of lung cancer

Day 30 discussion

Action: For discussion

Oncology

3.1.33. [Buparlisib - EMEA-003607-PIP01-24](#)

Treatment of head and neck epithelial malignant neoplasms

Day 30 discussion

Action: For discussion

Oncology

3.1.34. [Gedatolisib - EMEA-003612-PIP01-24](#)

Treatment of children with solid malignancies / Treatment of haematologic malignancies /
Treatment of central nervous system malignancies

Day 30 discussion

Action: For discussion

Oncology

3.1.35. Ifinatamab deruxtecan - Orphan - EMEA-003613-PIP01-24

Daiichi Sankyo Europe GmbH; Treatment of lung cancer

Day 30 discussion

Action: For discussion

Oncology

3.1.36. Letetresgene autoleucel - Orphan - EMEA-002476-PIP03-24

Adaptimmune B. V.; Treatment of soft tissue sarcoma

Day 30 discussion

Action: For discussion

Oncology

3.1.37. Rocatinlimab - EMEA-002886-PIP03-24

Treatment of asthma

Day 30 discussion

Action: For discussion

Pneumology - Allergology

3.1.38. Sargramostim - EMEA-003568-PIP02-24

Treatment of autoimmune pulmonary alveolar proteinosis (aPAP)

Day 30 discussion

Action: For discussion

Pneumology - Allergology

3.1.39. Iloperidone - EMEA-000995-PIP02-24

Treatment of bipolar disorder

Day 30 discussion

Action: For discussion

Psychiatry

3.1.40. Purified antigen fractions of inactivated split virion influenza virus type A, H1N1 / influenza virus type A, H3N2 / influenza virus type B, Victoria lineage - EMEA-003641-PIP01-24

Prevention of influenza infection

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. Lomitapide - EMEA-C1-001124-PIP01-10-M05

Amryt Pharmaceuticals DAC; Treatment of heterozygous and homozygous familial hypercholesterolaemia

Day 30 discussion

Action: For discussion

Endocrinology-Gynaecology-Fertility-Metabolism

3.2.2. Brexpiprazole - EMEA-C3-001185-PIP01-11-M08

Otsuka Pharmaceutical Development & commercialisation Europe GmbH; Treatment of schizophrenia

Day 30 discussion

Action: For discussion

Psychiatry

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

Seqirus Netherlands B.V.; Prevention of influenza infection

Day 30 discussion

Action: For discussion

Vaccines

3.3. Discussions on Modification of an Agreed Paediatric Investigation Plan

3.3.1. Gadopiclenol - EMEA-001949-PIP01-16-M07

Guerbet; Detection and visualisation of disorders or lesions with suspected abnormal

vascularity in various body regions for diagnostic purposes

Day 30 discussion

Action: For discussion

Diagnostic

3.3.2. [Gadopiclenol - EMEA-001949-PIP02-18-M05](#)

Guerbet; Detection and visualisation of disorders or lesions with suspected abnormal vascularity in various body regions for diagnostic purposes

Day 30 discussion

Action: For discussion

Diagnostic

3.3.3. [Crinecerfont - Orphan - EMEA-002700-PIP01-19-M02](#)

Neurocrine Therapeutics Ltd.; Treatment of congenital adrenal hyperplasia

Day 30 discussion

Action: For discussion

Endocrinology-Gynaecology-Fertility-Metabolism

3.3.4. [Humanised monoclonal antibody Fab fragment - Orphan - EMEA-003253-PIP01-22-M01](#)

Sanofi B.V; Treatment of achondroplasia

Day 30 discussion

Action: For discussion

Endocrinology-Gynaecology-Fertility-Metabolism

3.3.5. [Navepegritide - Orphan - EMEA-002689-PIP02-23-M01](#)

Ascendis Pharma Growth Disorders A/S; Treatment of achondroplasia

Day 30 discussion

Action: For discussion

Endocrinology-Gynaecology-Fertility-Metabolism

3.3.6. [Teplizumab - EMEA-000524-PIP01-08-M03](#)

Sanofi Winthrop Industrie; Treatment of type 1 diabetes mellitus

Day 30 discussion

Action: For discussion

Endocrinology-Gynaecology-Fertility-Metabolism

3.3.7. [Survodutide - EMEA-002942-PIP02-20-M02](#)

Boehringer Ingelheim International GmbH; Treatment of obesity

Day 30 discussion

Action: For discussion

Gastroenterology-Hepatology

3.3.8. [Tezepelumab - EMEA-001613-PIP03-21-M01](#)

AstraZeneca AB; Treatment of eosinophilic esophagitis

Day 30 discussion

Action: For discussion

Gastroenterology-Hepatology

3.3.9. [Vonoprazan - EMEA-002703-PIP01-19-M01](#)

Phathom Pharmaceuticals, Inc.; Treatment of gastroesophageal reflux disease (GORD) /
Treatment of *Helicobacter pylori* infection

Day 30 discussion

Action: For discussion

Gastroenterology-Hepatology

3.3.10. [Fitusiran - Orphan - EMEA-001855-PIP01-15-M06](#)

Sanofi B.V.; Treatment of haemophilia B / Treatment of haemophilia A

Day 30 discussion

Action: For discussion

Haematology-Hemostaseology

3.3.11. [Osivelotor - EMEA-003241-PIP01-22-M01](#)

Pfizer Europe MA EEIG; Treatment of sickle cell disease

Day 30 discussion

Action: For discussion

Haematology-Hemostaseology

3.3.12. [Narsoplimab - Orphan - EMEA-002479-PIP01-18-M02](#)

Omeros Ireland Limited; Treatment in haematopoietic stem cell transplantation

Day 30 discussion

Action: For discussion

Haematology-Hemostaseology

3.3.13. [Vadadustat - EMEA-001944-PIP01-16-M05](#)

Medice Arzneimittel Pütter GmbH & Co KG; Treatment of anaemia due to chronic disorders

Day 30 discussion

Action: For discussion

Haematology-Hemostaseology

3.3.14. [Allogeneic bone marrow derived mesenchymal stromal cells, ex-vivo expanded - Orphan - EMEA-002706-PIP01-19-M03](#)

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

Day 30 discussion

Action: For discussion

Immunology-Rheumatology-Transplantation

3.3.15. [Human normal immunoglobulin - EMEA-001290-PIP01-12-M01](#)

LFB Biotechnologies; Treatment of primary immunodeficiency (PID)

Day 30 discussion

Action: For discussion

Immunology-Rheumatology-Transplantation

3.3.16. [Ceftobiprole medocaril sodium - EMEA-000205-PIP02-11-M07](#)

Basilea Pharmaceutica Deutschland GmbH; Treatment of pneumonia

Day 30 discussion

Action: For discussion

Infectious Diseases

3.3.17. [Lenacapavir sodium - EMEA-002740-PIP01-19-M01](#)

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

Day 30 discussion

Action: For discussion

Infectious Diseases

3.3.18. Pretomanid - Orphan - EMEA-002115-PIP01-17-M06

Global Alliance for TB Drug Development; Treatment of multi-drug-resistant tuberculosis

Day 30 discussion

Action: For discussion

Infectious Diseases

3.3.19. Rilpivirine / dolutegravir - EMEA-001750-PIP01-15-M07

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

Day 30 discussion

Action: For discussion

Infectious Diseases

3.3.20. Tenofovir alafenamide / emtricitabine / cobicistat / darunavir - EMEA-001825-PIP01-15-M05

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

Day 30 discussion

Action: For discussion

Infectious Diseases

3.3.21. Cenobamate - EMEA-002563-PIP02-19-M03

Angelini Pharma S.p.A; Treatment of focal onset seizures (FOS) / Treatment of primary generalised tonic clonic seizures (PGTC)

Day 30 discussion

Action: For discussion

Neurology

3.3.22. Dexamethasone sodium phosphate encapsulated in human autologous erythrocytes - Orphan - EMEA-001957-PIP02-19-M01

Quince Therapeutics, S.p.A.; Treatment of ataxia telangiectasia

Day 30 discussion

Action: For discussion

Neurology

3.3.23. Efgartigimod alfa - Orphan - EMEA-002597-PIP01-19-M02

argenx BV; Treatment of generalised myasthenia gravis

Day 30 discussion

Action: For discussion

Neurology

3.3.24. 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-M01

AbbVie Limited; Treatment of vanishing white matter disease

Day 30 discussion

Action: For discussion

Neurology

3.3.25. Midostaurin - Orphan - EMEA-000780-PIP01-09-M08

Novartis Europharm Limited; Treatment of acute myeloid leukaemia / Treatment of malignant mastocytosis / Treatment of mast cell leukaemia

Day 30 discussion

Action: For discussion

Oncology

3.3.26. Venetoclax - Orphan - EMEA-002018-PIP02-16-M06

AbbVie Ltd; Treatment of haematopoietic and lymphoid malignant neoplasms / Treatment of solid tumour malignant neoplasms

Day 30 discussion

Action: For discussion

Oncology

3.3.27. Gilteritinib (as fumarate) - Orphan - EMEA-002064-PIP01-16-M06

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

Day 30 discussion

Action: For discussion

Oncology / Haematology-Hemostaseology

3.3.28. Bupropion hydrochloride / naltrexone hydrochloride - EMEA-001373-PIP01-12-M06

Orexigen Therapeutics Ireland Limited; Treatment of obesity

Day 30 discussion

Action: For discussion

Other

3.3.29. Ketamine / sufentanil - EMEA-001739-PIP02-16-M03

Cessatech A/S; Treatment of acute pain

Day 30 discussion

Action: For discussion

Pain

3.3.30. Meloxicam / bupivacaine - EMEA-002246-PIP01-17-M05

Heron Therapeutics B.V.; Treatment of acute postoperative pain

Day 30 discussion

Action: For discussion

Pain

3.3.31. Benralizumab - EMEA-001214-PIP04-19-M01

AstraZeneca AB; Treatment of hypereosinophilic syndrome

Day 30 discussion

Action: For discussion

Pneumology - Allergology

3.3.32. Donidalorsen - Orphan - EMEA-003112-PIP01-21-M01

Otsuka Pharmaceutical Netherlands B.V.; Treatment of hereditary angioedema

Day 30 discussion

Action: For discussion

Pneumology - Allergology

3.3.33. COVID-19 Vaccine (ChAdOx1-S [recombinant]) - EMEA-002862-PIP01-20-M04

AstraZeneca AB; Prevention of coronavirus disease 2019 (COVID-19)

Day 30 discussion

Action: For discussion

Vaccines

- 3.3.34. *Neisseria meningitidis* serogroup B protein-based active substance / recombinant *Neisseria meningitidis* serogroup B protein 3 / recombinant *Neisseria meningitidis* serogroup B protein 2 / recombinant *Neisseria meningitidis* serogroup B protein 1 / *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-003379-PIP01-22-M01
-

Sanofi Pasteur; Prevention of meningococcal disease (serogroups A, B, C, W and Y)

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 27 May 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. Fezolinetant - EMEA-04-2024

Astellas Pharma Europe B.V.; The classes of androgen receptor modulator, of oestrogen receptor modulator, of growth and sex hormone as well as their releasing or inhibiting factors, and of sex hormone-metabolism modulator medicinal products for treatment of breast malignant neoplasms.

Treatment of moderate to severe vasomotor symptoms in women with breast cancer on adjuvant endocrine therapy.

Action: For adoption

6.1.2. (PLGA-PEG-PSA) a ligand-presenting nanoparticle - EMEA-05-2024

Aviceda Therapeutics, Inc.; All classes of medicinal products for treatment of age-related macular degeneration and diabetic macular oedema.

Treatment of geographic atrophy secondary to age-related macular degeneration

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

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

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

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

PDCO member: Karen van Malderen

Action: For information

9.1.4. [Scientific Committee Meetings – alternating face-to-face and virtual meetings schedule for 2025](#)

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 Chair: Brian Aylward

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

9.4.2. European Directorate for the Quality of Medicines & HealthCare (EDQM)

Feedback from the Paediatric Formulary meeting held in Strasbourg (FR) on 5-6 March 2024

PDCO member: Siri Wang

Action: For information

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

10. Any other business

10.1. Changes to the labelling of benzyl alcohol in paediatric formulations

PDCO member: Karen Van Malderen

Action: For information

10.2. Real World Evidence, including DARWIN EU®

Scope: Feedback on the February 2024 patient registry workshop, including recommendations/actions, and impact on PDCO

Action: For information

11. Breakout sessions

11.1. Internal PDCO Operations

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

11.2. Paediatric oncology

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

11.3. Neonatology

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

11.4. HIV

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/