Paediatric Committee (PDCO)
Draft Agenda for the meeting on 25-28 June 2024

Chair: Brian Aylward – Vice-Chair: Sylvie Benchetrit
25 June 2024, 14:00 - 19:00, room 2C
26 June 2024, 08:30 - 19:00, room 2C
27 June 2024, 08:30 - 19:00, room 2C
28 June 2024, 08:30 - 13:00, room 2C

Health and safety information
In accordance with the Agency’s health and safety policy, delegates are to be briefed on health, safety and emergency information and procedures prior to the start of the meeting.

Disclaimers
Some of the information contained in this agenda is considered commercially confidential or sensitive and therefore not disclosed. With regard to intended therapeutic indications or procedure scopes listed against products, it must be noted that these may not reflect the full wording proposed by applicants and may also vary during the course of the review. Additional details on some of these procedures will be published in the PDCO Committee meeting reports (after the PDCO Opinion is adopted), and on the Opinions and decisions on paediatric investigation plans webpage (after the EMA Decision is issued).

Note on access to documents
Some documents mentioned in the agenda cannot be released at present following a request for access to documents within the framework of Regulation (EC) No 1049/2001 as they are subject to 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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9.2.2. Pharmacovigilance Risk Assessment Committee (PRAC)

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10.2. Potential pitfalls from uncritical use of artificial intelligence (AI)

10.3. Practical aspects of IRIS for PDCO Members

10.4. Presenting efforts/work ongoing on a facilitation framework

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11.2. Neonatology

11.3. HIV
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 25-28 June 2024. See June 2024 PDCO minutes (to be published post July PDCO meeting).

1.2. **Adoption of agenda**

PDCO agenda for 25-28 June 2024.

1.3. **Adoption of the minutes**

PDCO minutes for 28-31 May 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. **Transglutaminase 2 inhibitor - EMEA-003513-PIP01-23**

Treatment of coeliac disease

Day 120 opinion

**Action**: For adoption

Gastroenterology-Hepatology

2.1.2. **Mezagitamab - EMEA-003502-PIP01-23**

Treatment of immune thrombocytopenia

Day 120 opinion

**Action**: For adoption

Haematology-Hemostaseology

2.1.3. **Ganaxolone - EMEA-002341-PIP03-23**

Treatment of refractory status epilepticus

Day 120 opinion
2.1.4. **Vosoritide - EMEA-002033-PIP02-23**

Treatment of hypochondroplasia

Day 120 opinion

**Action:** For adoption

Other

2.1.5. **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 120 opinion

**Action:** For adoption

Vaccines

2.1.6. **Tadalafil / ambrisentan - EMEA-003617-PIP01-24**

Treatment of pulmonary arterial hypertension

Day 60 opinion

**Action:** For adoption

Cardiovascular Diseases

2.1.7. **Tadalafil / ambrisentan - EMEA-003621-PIP01-24**

Treatment of pulmonary arterial hypertension

Day 60 opinion

**Action:** For adoption

Cardiovascular Diseases

2.1.8. **Tarumase - EMEA-003616-PIP01-24**

Treatment of chronic wounds

Day 60 opinion

**Action:** For adoption

Dermatology
2.1.9. Copper ($^{64}$Cu) oxodotreotide - Orphan - EMEA-003610-PIP01-24

Curium Pet France; Diagnosis of neuroendocrine neoplasms

Day 60 opinion

**Action:** For adoption

Diagnostic

2.1.10. Alvelestat - EMEA-003605-PIP01-24

Treatment of alpha-1 antitrypsin deficiency

Day 60 opinion

**Action:** For adoption

Endocrinology-Gynaecology-Fertility-Metabolism

2.1.11. 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 60 opinion

**Action:** For adoption

Endocrinology-Gynaecology-Fertility-Metabolism

*Note: Withdrawal request received on 18 June 2024*

2.1.12. Inobrodib - EMEA-003622-PIP01-24

Treatment of multiple myeloma

Day 60 opinion

**Action:** For adoption

Haematology-Hemostaseology

2.1.13. Acasunlimab - EMEA-003606-PIP01-24

Treatment of lung cancer

Day 60 opinion

**Action:** For adoption

Oncology
2.1.14. **Buparlisib - EMEA-003607-PIP01-24**  
Treatment of head and neck epithelial malignant neoplasms  
Day 60 opinion  
**Action**: For adoption  
Oncology

2.1.15. **Ifinatamab deruxtecan - Orphan - EMEA-003613-PIP01-24**  
Daiichi Sankyo Europe GmbH; Treatment of lung cancer  
Day 60 opinion  
**Action**: For adoption  
Oncology

2.1.16. **Iloperidone - EMEA-000995-PIP02-24**  
Treatment of bipolar disorder  
Day 60 opinion  
**Action**: For adoption  
Psychiatry  
*Note: Withdrawal request received on 19 June 2024*

2.1.17. **Brukinsa - EMEA-002354-PIP03-24**  
Treatment of lymphoplasmacytic lymphoma / Treatment of mature B-cell neoplasms (excluding lymphocytic lymphoma)  
Day 30 opinion  
**Action**: For adoption  
Oncology

2.2. **Opinions on Compliance Check**

2.2.1. **Recombinant influenza hemagglutinin-strain A (H1N1 subtype) / recombinant influenza hemagglutinin-strain A (H3N2 subtype) / recombinant influenza hemagglutinin-strain B (RIV3) - EMEA-C-003640-PIP01-24**  
Sanofi Winthrop Industrie; Prevention of influenza disease  
Day 30 opinion  
**Action**: For adoption  
Vaccines
2.2.2. **Purified antigen fractions of inactivated split virion influenza virus type A, H1N1 / influenza virus type A, H3N2 / influenza virus type B, Victoria lineage - EMEA-C-003641-PIP01-24**

GlaxoSmithKline Biologicals S.A.; Prevention of influenza infection

Day 30 opinion

**Action**: For adoption

Vaccines

2.2.3. **Estetrol monohydrate / drospirenone - EMEA-C-001332-PIP01-12-M06**

Estetra SRL; Prevention of pregnancy

Day 30 opinion

**Action**: For adoption

Endocrinology-Gynaecology-Fertility-Metabolism

2.2.4. **Liraglutide - EMEA-C-000128-PIP02-09-M05**

Novo Nordisk A/S; Treatment of obesity

Day 30 opinion

**Action**: For adoption

Endocrinology-Gynaecology-Fertility-Metabolism

2.3. **Opinions on Modification of an Agreed Paediatric Investigation Plan**

2.3.1. **Gadopiclenol - EMEA-001949-PIP01-16-M07**

Guerbet; Detection and visualisation of areas with disruption of the blood brain barrier and/or abnormal vascularity for diagnostic purposes

Day 60 opinion

**Action**: For adoption

Diagnostic

2.3.2. **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 60 opinion

**Action**: For adoption

Diagnostic
2.3.3. Crinecerfont - Orphan - EMEA-002700-PIP01-19-M02

Neurocrine Therapeutics Ltd.; Treatment of congenital adrenal hyperplasia

Day 60 opinion

Action: For adoption

Endocrinology-Gynaecology-Fertility-Metabolism

2.3.4. Humanised monoclonal antibody Fab fragment - Orphan - EMEA-003253-PIP01-22-M01

Sanofi B.V; Treatment of achondroplasia

Day 60 opinion

Action: For adoption

Endocrinology-Gynaecology-Fertility-Metabolism

2.3.5. Navepegritide - Orphan - EMEA-002689-PIP02-23-M01

Ascendis Pharma Growth Disorders A/S; Treatment of achondroplasia

Day 60 opinion

Action: For adoption

Endocrinology-Gynaecology-Fertility-Metabolism

2.3.6. Teplizumab - EMEA-000524-PIP01-08-M03

Sanofi Winthrop Industrie; Treatment of type 1 diabetes mellitus

Day 60 opinion

Action: For adoption

Endocrinology-Gynaecology-Fertility-Metabolism

2.3.7. Survodutide - EMEA-002942-PIP02-20-M02

Boehringer Ingelheim International GmbH; Treatment of obesity

Day 60 opinion

Action: For adoption

Gastroenterology-Hepatology

2.3.8. Tezepelumab - EMEA-001613-PIP03-21-M01

AstraZeneca AB; Treatment of eosinophilic esophagitis

Day 60 opinion
**Action**: For adoption

**Gastroenterology-Hepatology**

### 2.3.9. Vonoprazan - EMEA-002703-PIP01-19-M01

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

Day 60 opinion

**Action**: For adoption

**Gastroenterology-Hepatology**

### 2.3.10. Fitusiran - Orphan - EMEA-001855-PIP01-15-M06

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

Day 60 opinion

**Action**: For adoption

**Haematology-Hemostaseology**

### 2.3.11. Osivelotor - EMEA-003241-PIP01-22-M01

Pfizer Europe MA EEIG; Treatment of sickle cell disease

Day 60 opinion

**Action**: For adoption

**Haematology-Hemostaseology**

### 2.3.12. Narsoplimab - Orphan - EMEA-002479-PIP01-18-M02

Omeros Ireland Limited; Treatment in haematopoietic stem cell transplantation

Day 60 opinion

**Action**: For adoption

**Haematology-Hemostaseology**

### 2.3.13. Vadadustat - EMEA-001944-PIP01-16-M05

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

Day 60 opinion

**Action**: For adoption

**Haematology-Hemostaseology**
2.3.14. 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 60 opinion

**Action:** For adoption

Immunology-Rheumatology-Transplantation

2.3.15. Human normal immunoglobulin - EMEA-001290-PIP01-12-M01

LFB Biotechnologies; Treatment of primary immunodeficiency (PID)

Day 60 opinion

**Action:** For adoption

Immunology-Rheumatology-Transplantation

2.3.16. Ceftobiprole medocaril sodium - EMEA-000205-PIP02-11-M07

Basilea Pharmaceutica Deutschland GmbH; Treatment of pneumonia

Day 60 opinion

**Action:** For adoption

Infectious Diseases

2.3.17. Lenacapavir sodium - EMEA-002740-PIP01-19-M01

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

Day 60 opinion

**Action:** For adoption

Infectious Diseases

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

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

Day 60 opinion

**Action:** For adoption

Infectious Diseases
### 2.3.19. Rilpivirine / dolutegravir - EMEA-001750-PIP01-15-M07

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

**Day 60 opinion**

**Action:** For adoption

Infectious Diseases

### 2.3.20. 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 60 opinion**

**Action:** For adoption

Infectious Diseases

### 2.3.21. 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 60 opinion**

**Action:** For adoption

Neurology

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

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

**Day 60 opinion**

**Action:** For adoption

Neurology

### 2.3.23. Efgartigimod alfa - Orphan - EMEA-002597-PIP01-19-M02

argenx BV; Treatment of generalised myasthenia gravis

**Day 60 opinion**

**Action:** For adoption

Neurology
2.3.24. Sodium \( ((2S)-1,4\text{-}bis[2\text{-}(4\text{-}chloro\text{-}3\text{-}fluorophenoxy})\text{acetamido}]\text{bicyclo}[2.2.2]\text{octan}-2\text{-}yl\text{)}\text{oxy})\text{methyl hydrogen phosphate}-2\text{-}amino-2\text{-}(hydroxymethyl)\text{propane}-1,3\text{-}diol (1/1/1) - Orphan - EMEA-003344-PIP01-22-M01

AbbVie Limited; Treatment of vanishing white matter disease

Day 60 opinion

**Action**: For adoption

Neurology

2.3.25. 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 60 opinion

**Action**: For adoption

Oncology

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

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

Day 60 opinion

**Action**: For adoption

Oncology

*Note: Withdrawal request received on 14 June 2024*

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

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

Day 60 opinion

**Action**: For adoption

Oncology / Haematology-Hemostaseology

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

Orexigen Therapeutics Ireland Limited; Treatment of obesity

Day 60 opinion

**Action**: For adoption

Other
2.3.29. Ketamine / sufentanil - EMEA-001739-PIP02-16-M03

Cessatech A/S; Treatment of acute pain
Day 60 opinion

**Action**: For adoption

Pain

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

Heron Therapeutics B.V.; Treatment of acute postoperative pain
Day 60 opinion

**Action**: For adoption

Pain

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

AstraZeneca AB; Treatment of hypereosinophilic syndrome
Day 60 opinion

**Action**: For adoption

Pneumology - Allergology

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

Otsuka Pharmaceutical Netherlands B.V.; Treatment of hereditary angioedema
Day 60 opinion

**Action**: For adoption

Pneumology - Allergology

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

AstraZeneca AB; Prevention of coronavirus disease 2019 (COVID-19)
Day 60 opinion

**Action**: For adoption

Vaccines

2.3.34. 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 60 opinion

**Action:** For adoption

Vaccines

2.3.35. **Osilodrostat - Orphan - EMEA-000315-PIP02-15-M04**

Recordati Rare Diseases SARL; Treatment of adrenal cortical hyperfunction

Day 30 opinion

**Action:** For adoption

Endocrinology-Gynaecology-Fertility-Metabolism

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. **Multivalent pneumococcal polysaccharide conjugate to carrier protein - EMEA-C1-002780-PIP02-20-M01**

Sanofi Pasteur; Prevention of disease caused by *Streptococcus pneumoniae*

Day 30 letter

**Action:** For information

Vaccines

2.7.2. **Selumetinib - EMEA-C2-001585-PIP01-13-M06**

AstraZeneca AB; Treatment of neurofibromatosis type 1
2.7.3. Olezarsen - EMEA-C1-003177-PIP01-21

Ionis Ireland Limited; Treatment of familial chylomicronaemia syndrome

2.7.4. Finerenone - EMEA-C1-001623-PIP03-20-M01

Bayer AG; Treatment of heart failure

2.7.5. Glucagon - EMEA-C2-001657-PIP01-14-M01

Amphastar France Pharmaceuticals; Treatment of hypoglycaemia

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. Seralutinib - Orphan - EMEA-002972-PIP02-23

Gossamer Bio 002 Limited; Treatment of pulmonary arterial hypertension

Day 90 discussion

Action: For discussion
Cardiovascular Diseases
3.1.2. Pegtibatinase - Orphan - EMEA-003518-PIP01-23
Travere Therapeutics Ireland Limited; Treatment of classical homocystinuria
Day 90 discussion
**Action**: For discussion
Endocrinology-Gynaecology-Fertility-Metabolism

3.1.3. Efinopegdutide - EMEA-003549-PIP01-23
Treatment of non-alcoholic steatohepatitis
Day 90 discussion
**Action**: For discussion
Gastroenterology-Hepatology

3.1.4. Hydroxycarbamide - EMEA-003388-PIP01-23
Treatment of sickle cell disease
Day 90 discussion
**Action**: For discussion
Haematology-Hemostaseology

3.1.5. Recombinant humanised IgG1, kappa light chain, long-acting monoclonal antibody - EMEA-003510-PIP01-23
Prevention of hereditary angioedema attacks
Day 90 discussion
**Action**: For discussion
Haematology-Hemostaseology

3.1.6. Belumosudil - Orphan - EMEA-003425-PIP02-23
Sanofi Winthrop Industrie; Treatment of chronic lung allograft dysfunction
Day 90 discussion
**Action**: For discussion
Immunology-Rheumatology-Transplantation

3.1.7. Iptacopan - EMEA-002705-PIP06-23
Treatment of myasthenia gravis
Day 90 discussion
Action: For discussion

Neurology

3.1.8. Adeno-associated viral vector serotype 8 containing the 3' human otoferlin coding sequence / adeno-associated viral vector serotype 8 containing the 5' human otoferlin coding sequence - Orphan - EMEA-003524-PIP01-23

Sensorion SA; Treatment of otoferlin gene-mediated hearing loss
Day 90 discussion

Action: For discussion

Other

3.1.9. Mannose-1-phosphate - Orphan - EMEA-003460-PIP01-23

Glycomine Inc; Treatment of phosphomannomutase 2-congenital disorder of glycosylation
Day 90 discussion

Action: For discussion

Other

3.1.10. Retatrutide - EMEA-003258-PIP02-23

Treatment of obesity
Day 90 discussion

Action: For discussion

Other

3.1.11. Rilonacept - Orphan - EMEA-003571-PIP01-23

Kiniksa Pharmaceuticals (UK), Ltd.; Treatment of idiopathic pericarditis
Day 90 discussion

Action: For discussion

Other / Cardiovascular Diseases

3.1.12. Luliconazole - EMEA-003604-PIP01-24

Treatment of onychomycosis
Day 60 discussion

Action: For discussion

Dermatology
3.1.13. 2-Isopropyl-3H-naphtho[1,2-d]imidazole-4,5-dione - Orphan - EMEA-003618-PIP01-24

Abliva AB; Treatment of primary mitochondrial disease
Day 60 discussion
**Action**: For discussion
Endocrinology-Gynaecology-Fertility-Metabolism


Soleno Therapeutics Europe Ltd.; Treatment of Prader-Willi syndrome
Day 60 discussion
**Action**: For discussion
Endocrinology-Gynaecology-Fertility-Metabolism / Psychiatry / Neurology

3.1.15. Pegozafermin - EMEA-003619-PIP01-24

Treatment of metabolic dysfunction-associated steatohepatitis (MASH)
Day 60 discussion
**Action**: For discussion
Gastroenterology-Hepatology

3.1.16. Telitacicept - EMEA-002824-PIP02-24

Treatment of myasthenia gravis
Day 60 discussion
**Action**: For discussion
Immunology-Rheumatology-Transplantation

3.1.17. 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 60 discussion
**Action**: For discussion
Immunology-Rheumatology-Transplantation / Haematology-Hemostaseology

3.1.18. EMEA-003609-PIP01-24

Treatment of respiratory tract disease caused by human respiratory syncytial virus
Day 60 discussion

**Action**: For discussion

Infectious Diseases

### 3.1.19. EMEA-003306-PIP02-24

Treatment of human respiratory syncytial virus (RSV)

Day 60 discussion

**Action**: For discussion

Infectious Diseases

### 3.1.20. EMEA-003615-PIP01-24

Treatment of Prader Willi syndrome

Day 60 discussion

**Action**: For discussion

Neurology

### 3.1.21. Frexalimab - EMEA-002945-PIP04-24

Treatment of multiple sclerosis

Day 60 discussion

**Action**: For discussion

Neurology

### 3.1.22. Gedatolisib - EMEA-003612-PIP01-24

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

Day 60 discussion

**Action**: For discussion

Oncology

### 3.1.23. Letetresgene autoleucel - Orphan - EMEA-002476-PIP03-24

Adaptimmune B. V.; Treatment of soft tissue sarcoma

Day 60 discussion

**Action**: For discussion

Oncology
3.1.24. **Rocatinlimab - EMEA-002886-PIP03-24**

Treatment of asthma

Day 60 discussion

**Action**: For discussion

Pneumology - Allergology

3.1.25. **Sargramostim - EMEA-003568-PIP02-24**

Treatment of autoimmune pulmonary alveolar proteinosis (aPAP)

Day 60 discussion

**Action**: For discussion

Pneumology - Allergology

3.1.26. **Adeno-associated virus serotype 9 vector containing the human LAMP2 isoform B transgene - Orphan - EMEA-003639-PIP01-24**

Rocket Pharmaceuticals, B.V.; Treatment of Danon disease

Day 30 discussion

**Action**: For discussion

Cardiovascular Diseases

3.1.27. **Zerlasiran - EMEA-003627-PIP01-24**

Atherosclerotic cardiovascular disease

Day 30 discussion

**Action**: For discussion

Cardiovascular Diseases

3.1.28. **EMEA-003632-PIP01-24**

Treatment of symptomatic heart failure

Day 30 discussion

**Action**: For discussion

Cardiovascular Diseases

3.1.29. **EMEA-003633-PIP01-24**

Treatment of psoriasis

Day 30 discussion
**Action**: For discussion

**Dermatology**

3.1.30. **Rememulgene arelactibac - EMEA-003638-PIP01-24**

Treatment of diabetic foot ulcer / Treatment of venous leg ulcer

Day 30 discussion

**Action**: For discussion

**Dermatology**

3.1.31. **Dasiglucagon - Orphan - EMEA-002233-PIP03-24**

Zealand Pharma A/S; Treatment of congenital hyperinsulinism

Day 30 discussion

**Action**: For discussion

**Endocrinology-Gynaecology-Fertility-Metabolism**

3.1.32. **Etavopivat - Orphan - EMEA-002924-PIP03-24**

Novo Nordisk A/S; Treatment of thalassemia

Day 30 discussion

**Action**: For discussion

**Haematology-Hemostaseology**

3.1.33. **Sonelokimab - EMEA-002568-PIP03-24**

Treatment of active psoriatic arthritis / Treatment of chronic idiopathic arthritis (including rheumatoid arthritis, spondyloarthritis, psoriatic arthritis and juvenile idiopathic arthritis)

Day 30 discussion

**Action**: For discussion

**Immunology-Rheumatology-Transplantation**

3.1.34. **Heterologous intestinal microbiota from healthy donors - EMEA-003635-PIP01-24**

Treatment of *Clostridioides difficile* infection (CDI)

Day 30 discussion

**Action**: For discussion

**Infectious Diseases**
3.1.35.  EMEA-003602-PIP01-24

Treatment of tuberculosis
Day 30 discussion
Action: For discussion
Infectious Diseases

3.1.36.  Adeno-associated viral vector serotype 9 containing the human MECP2 gene - Orphan - EMEA-003634-PIP01-24

Taysha Gene Therapies, Inc.; Treatment of Rett syndrome
Day 30 discussion
Action: For discussion
Neurology

3.1.37.  Aldesleukin - Orphan - EMEA-001556-PIP02-24

ILTOO Pharma SAS; Treatment of amyotrophic lateral sclerosis (ALS)
Day 30 discussion
Action: For discussion
Neurology


NovaCurie GmbH; Treatment of low-grade glioma
Day 30 discussion
Action: For discussion
Neurology

3.1.39.  EMEA-003631-PIP01-24

Treatment of all conditions included in the category of malignant neoplasms (except haematopoietic and lymphoid tissue) whose treatment may include DNA-damaging agents
Day 30 discussion
Action: For discussion
Oncology
3.1.40. **Anitocabtagene autoleucel - EMEA-003628-PIP01-24**

Treatment of multiple myeloma

Day 30 discussion

**Action**: For discussion

**Oncology**

3.1.41. **Golcadomide - Orphan - EMEA-003161-PIP02-24**

Bristol-Myers Squibb Pharma EEIG; Treatment of mature B cell neoplasms

Day 30 discussion

**Action**: For discussion

**Oncology**

3.1.42. **Tafasitamab - Orphan - EMEA-002499-PIP03-24**

Incyte Biosciences Distribution B.V; Treatment of mature B-cell neoplasms [excluding diffuse large B-cell lymphoma (DLBCL)]

Day 30 discussion

**Action**: For discussion

**Oncology**

3.1.43. **Esonadogene imvoparvovec - Orphan - EMEA-003626-PIP01-24**

Neurophth Therapeutics, Inc.; Treatment of Leber's hereditary optic neuropathy

Day 30 discussion

**Action**: For discussion

**Ophthalmology**

3.1.44. **Tacrolimus - EMEA-003625-PIP01-24**

Treatment of vernal keratoconjunctivitis

Day 30 discussion

**Action**: For discussion

**Ophthalmology**

3.1.45. **Rupatadine / montelukast - EMEA-003629-PIP01-24**

Treatment of allergic rhinitis

Day 30 discussion
Action: For discussion

Oto-rhino-laryngology

3.1.46. Human metapneumovirus, virus-like particle / human respiratory syncytial virus, virus-like particle - EMEA-003636-PIP01-24

Prevention of lower respiratory tract disease caused by respiratory syncytial virus or human metapneumovirus

Day 30 discussion

Action: For discussion

Vaccines

3.1.47. mRNA-based vaccine consisting of 3 mRNAs encoding the VP1 capsid protein from NoV genotypes, GII.4, GII.3 and GI.3 - EMEA-003637-PIP01-24

Prevention of norovirus acute gastroenteritis

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. Sebetralstat - EMEA-C1-002723-PIP01-19-M02

KalVista Pharmaceuticals Ltd; Treatment of hereditary angioedema

Day 30 discussion

Action: For discussion

Haematology-Hemostaseology

3.2.2. Emtricitabine / tenofovir alafenamide - EMEA-C1-001577-PIP02-14-M05

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

Day 30 discussion

Action: For discussion

Infectious Diseases
3.2.3. Palbociclib - EMEA-C-002146-PIP01-17-M04

Pfizer Europe MA EEIG; Treatment of Ewing sarcoma
Day 30 discussion
Action: For discussion
Oncology

3.3. Discussions on Modification of an Agreed Paediatric Investigation Plan

3.3.1. Fenebrutinib - EMEA-002349-PIP03-20-M01

Roche Registration GmbH; Treatment of multiple sclerosis
Day 30 discussion
Action: For discussion

3.3.2. Ambrisentan - Orphan - EMEA-000434-PIP01-08-M10

GlaxoSmithKline (Ireland) Limited; Treatment pulmonary arterial hypertension
Day 30 discussion
Action: For discussion
Cardiovascular Diseases

3.3.3. Spesolimab - EMEA-002475-PIP03-22-M01

Boehringer Ingelheim International GmbH; Treatment of Netherton syndrome
Day 30 discussion
Action: For discussion
Dermatology

3.3.4. Macimorelin acetate - EMEA-001988-PIP01-16-M02

Atnahs Pharma Netherlands B. V.; Diagnosis of growth hormone deficiency
Day 30 discussion
Action: For discussion
Diagnostic

3.3.5. Apremilast - EMEA-000715-PIP02-11-M08

Amgen Europe B.V.; Treatment of juvenile idiopathic arthritis (JIA) / Treatment of juvenile psoriatic arthritis (JPsA)
Day 30 discussion

**Action:** For discussion

Immunology-Rheumatology-Transplantation

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### 3.3.6. Apremilast - EMEA-000715-PIP05-13-M06

Amgen Europe B.V; Treatment of Behçet's disease

Day 30 discussion

**Action:** For discussion

Immunology-Rheumatology-Transplantation

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### 3.3.7. Deucravacitinib - EMEA-002350-PIP02-20-M01

Bristol-Myers Squibb Pharma 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

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### 3.3.8. Human plasma derived C1-inhibitor (OCTA-C1-INH) - EMEA-002818-PIP01-20-M01

Octapharma Pharmazeutika Produktionsges. m.b.H; Treatment of hereditary angioedema (HAE)

Day 30 discussion

**Action:** For discussion

Immunology-Rheumatology-Transplantation

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### 3.3.9. Cefiderocol - EMEA-002133-PIP01-17-M04

Shionogi B.V.; Treatment of infections due to aerobic gram-negative bacteria

Day 30 discussion

**Action:** For discussion

Infectious Diseases

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### 3.3.10. Rezafungin acetate - Orphan - EMEA-002319-PIP01-17-M03

Mundipharma GmbH; Treatment of invasive candidiasis

Day 30 discussion

**Action:** For discussion
3.3.11. Sotrovimab - EMEA-002899-PIP01-20-M03

GlaxoSmithKline Trading Services Ltd; Treatment of coronavirus disease 2019 (COVID-19)

Day 30 discussion

Action: For discussion

Infectious Diseases

3.3.12. Tecovirimat monohydrate - Orphan - EMEA-001205-PIP02-19-M03

SIGA Technologies, Inc.; Treatment of the following viral infections in adults and children with body weight at least 13 kg: smallpox, monkeypox, cowpox. Also indicated to treat complications due to replication of vaccinia virus following vaccination against smallpox in adults and children with body weight at least 13 kg.

Day 30 discussion

Action: For discussion

Infectious Diseases

3.3.13. Lasmiditan - EMEA-002166-PIP01-17-M08

Eli Lilly and Company Ltd; Treatment of migraine headaches

Day 30 discussion

Action: For discussion

Neurology

3.3.14. Rimegepant - EMEA-002812-PIP02-20-M03

Pfizer Europe MA EEIG; Treatment of migraine headaches

Day 30 discussion

Action: For discussion

Neurology

3.3.15. Tasimelteon - Orphan - EMEA-001531-PIP01-13-M06

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

Day 30 discussion

Action: For discussion

Neurology
### 3.3.16. Vamorolone - Orphan - EMEA-001794-PIP02-16-M08

Santhera Pharmaceuticals (Deutschland) GmbH; Treatment of Duchenne muscular dystrophy

Day 30 discussion

**Action:** For discussion

Neurology

*Note: Withdrawal request received on 13 June 2024*

### 3.3.17. Vesleteplirsen - EMEA-003305-PIP01-22-M01

Sarepta Therapeutics Ireland; Treatment of Duchenne muscular dystrophy

Day 30 discussion

**Action:** For discussion

Neurology

### 3.3.18. Avelumab - EMEA-001849-PIP02-15-M05

Merck Healthcare KGaA; Treatment of malignant neoplasms of the central nervous system

Day 30 discussion

**Action:** For discussion

Oncology

### 3.3.19. Daratumumab - Orphan - EMEA-002152-PIP01-17-M04

Janssen-Cilag International NV; Treatment of lymphoid malignancies (except mature B-cell neoplasms)

Day 30 discussion

**Action:** For discussion

Oncology

### 3.3.20. Gemtuzumab ozogamicin - Orphan - EMEA-001733-PIP02-15-M03

Pfizer Europe MA EEIG; Treatment of acute myeloid leukaemia

Day 30 discussion

**Action:** For discussion

Oncology

### 3.3.21. Zamtocabtagene autoleucel - Orphan - EMEA-003009-PIP01-21-M01

Miltenyi Biomedicine GmbH; Treatment of mature B cell neoplasms
Day 30 discussion

**Action**: For discussion

**Oncology**

### 3.3.22. Patiromer sorbitex calcium - EMEA-001720-PIP01-14-M04

Vifor Fresenius Medical Care Renal Pharma France; Treatment of hyperkalaemia

Day 30 discussion

**Action**: For discussion

**Other**

### 3.3.23. Iptacopan - Orphan - EMEA-002705-PIP03-20-M01

Novartis Europharm Limited; Treatment of paroxysmal nocturnal haemoglobinuria (PNH)

Day 30 discussion

**Action**: For discussion

**Other / Haematology-Hemostaseology**

### 3.3.24. Ebola Zaire vaccine (rVSVΔG-ZEBOV-GP, live) - EMEA-001786-PIP01-15-M04

Merck Sharp & Dohme (Europe), Inc.; Prevention of Ebola disease

Day 30 discussion

**Action**: For discussion

**Vaccines**

### 3.3.25. Multivalent pneumococcal polysaccharide conjugate to carrier protein - EMEA-002780-PIP02-20-M02

Sanofi Pasteur; Prevention of disease caused by *Streptococcus pneumoniae*

Day 30 discussion

**Action**: For discussion

**Vaccines**

### 3.3.26. Recombinant varicella zoster virus glycoprotein E - EMEA-001426-PIP01-13-M03

GlaxoSmithKline Biologicals; Prevention of varicella zoster virus (VZV) reactivation

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 08 July 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. **Depemokimab - EMEA-06-2024**

GlaxoSmithKline Trading Services Limited; All classes of medicinal products for treatment of chronic obstructive pulmonary disease (COPD) (excluding chronic lung diseases associated with long-term airflow limitation, such as asthma, bronchopulmonary dysplasia, primary cilia dyskinesia, obstructive lung disease related to graft-versus-host disease (bone-marrow) transplantation.

Add-on to inhaled corticosteroid-based maintenance therapy for COPD patients.

**Action:** For adoption

6.1.2. **Leuprorelin - EMEA-07-2024**

Recordati Industria Chimica e Farmaceutica S.p.A.; 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 prostate malignant neoplasms.

Treatment of hormone dependent advanced prostate cancer and for the treatment of high-risk localised and locally advanced hormone dependent prostate cancer in combination with radiotherapy.

**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)**

No item

#### 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. Pharmacovigilance Risk Assessment Committee (PRAC)

Scope: Committee consultation of the PRAC-approved final revision of GVP Module XVI on risk minimisation measures post-public consultation

Action: For information

9.2.3. Joint resolution on the principles of conduct within EMA Scientific Committees, WPs and CMDx

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


Action: For information

9.3.4. Upcoming Innovation Task Force (ITF) meetings

Action: For information

9.3.5. Cardiovascular Working Party (CVS WP)

Scope: Under the supervision of the CVS WP a temporary drafting group (DG) has been established. The DG is working on the revision of the Paediatric Addendum on pulmonary arterial hypertension. Two PDCO representatives have been nominated to contribute to the DG.

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. Feedback and status update from the PaedForm Project (EDQM)

Scope: Feedback from the Paediatric Formulary meeting held in Strasbourg (FR) on 5-6 March 2024 and current status

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

Q2/2024 Update of the Business Pipeline report for the human scientific committees

Action: For information

10. Any other business

10.1. Health Technology Assessment body (HTAb) / EMA workshop on uncertainty management

Scope: To inform the Committee on an upcoming HTAb / EMA workshop series on uncertainty management

Action: For information

10.2. Potential pitfalls from uncritical use of artificial intelligence (AI)

Action: For information

10.3. Practical aspects of IRIS for PDCO Members

Scope: The PDCO will be informed about the practical aspects associated with the onboarding of paediatric procedures in IRIS
**10.4. Presenting efforts/work ongoing on a facilitation framework**

Scope: The PDCO will be informed about ongoing considerations towards building a paediatric drug development facilitation framework

**Action: For information**

**10.5. Real World Evidence, including DARWIN EU®**

**Action: For information**

**11. Breakout sessions**

**11.1. Paediatric oncology**

**Action: For discussion on Tuesday, 13:00 - 14:00, room 2C**

**11.2. Neonatology**

**Action: For discussion on Wednesday, 13:00 - 14:00, room 2C**

**11.3. HIV**

**Action: For discussion on Thursday, 13:00 - 14:00, room 2C**
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](https://www.ema.europa.eu/)

More detailed information on the above terms can be found on the EMA website: [www.ema.europa.eu/](https://www.ema.europa.eu/)