



EUROPEAN MEDICINES AGENCY  
SCIENCE MEDICINES HEALTH

13 January 2023  
EMA/PDCO/2399/2023  
Human Medicines Division

## Paediatric Committee (PDCO)

### Draft Agenda for the meeting on 17-20 January 2023

Chair: Brian Aylward – Vice-Chair: Sylvie Benchetrit

17 January 2023, 14:00 - 19:00, Virtual meeting

18 January 2023, 08:30 - 19:00, Virtual meeting

19 January 2023, 08:30 - 19:00, Virtual meeting

20 January 2023, 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 17-20 January 2023. See January 2023 PDCO minutes (to be published post February 2023 PDCO meeting).

### 1.2. Adoption of agenda

PDCO agenda for 17-20 January 2023.

### 1.3. Adoption of the minutes

PDCO minutes for 13-16 December 2022.

## 2. Opinions

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

### 2.1. Opinions on Products

#### 2.1.1. Zilebesiran - EMEA-003218-PIP01-22

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Treatment of hypertension

Day 120 opinion

**Action:** For adoption

Cardiovascular Diseases

#### 2.1.2. Danuglipron - EMEA-002944-PIP02-22

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Treatment of obesity

Day 120 opinion

**Action:** For adoption

Endocrinology-Gynaecology-Fertility-Metabolism

### 2.1.3. Insulin lispro - EMEA-003166-PIP01-21

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

Day 120 opinion

**Action:** For adoption

Endocrinology-Gynaecology-Fertility-Metabolism

### 2.1.4. EMEA-003241-PIP01-22

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Treatment of sickle cell disease

Day 120 opinion

**Action:** For adoption

Haematology-Hemostaseology

### 2.1.5. Cemdisiran (sodium) - Orphan - EMEA-003237-PIP01-22

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Regeneron Ireland DAC; Treatment of myasthenia gravis

Day 120 opinion

**Action:** For adoption

Neurology

### 2.1.6. Pozelimab - EMEA-003238-PIP01-22

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Treatment of myasthenia gravis

Day 120 opinion

**Action:** For adoption

Neurology

### 2.1.7. Lutetium (177Lu) edotreotide - Orphan - EMEA-003245-PIP01-22

---

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

Day 120 opinion

**Action:** For adoption

Oncology

### 2.1.8. Obecabtagene autoleucel - Orphan - EMEA-003171-PIP01-21

---

Autolus GmbH; Treatment of acute lymphoblastic leukaemia

Day 120 opinion

**Action:** For adoption

Oncology

#### 2.1.9. [Humanised IgG2 monoclonal antibody against interleukin-6 - EMEA-003215-PIP01-22](#)

---

Treatment of macular oedema

Day 120 opinion

**Action:** For adoption

Ophthalmology

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

---

Prevention of cardiovascular events in patients with atherosclerosis

Day 60 opinion

**Action:** For adoption

Cardiovascular Diseases / Uro-nephrology

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

---

Reneo Pharmaceuticals Inc; Treatment of primary mitochondrial disorders

Day 60 opinion

**Action:** For adoption

Endocrinology-Gynaecology-Fertility-Metabolism

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

---

Treatment of cutaneous lupus erythematosus

Day 60 opinion

**Action:** For adoption

Immunology-Rheumatology-Transplantation / Dermatology

#### 2.1.13. [Pembrolizumab - EMEA-001474-PIP03-22](#)

---

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

Day 60 opinion

**Action:** For adoption

---

Oncology

#### 2.1.14. Sotorasib - EMEA-002690-PIP02-22

Treatment of malignant colorectal neoplasms

Day 60 opinion

**Action:** For adoption

Oncology

#### 2.1.15. Upifitamab rilsodotin - EMEA-003340-PIP01-22

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

Day 60 opinion

**Action:** For adoption

Oncology

#### 2.1.16. Camlipixant - EMEA-003334-PIP01-22

Treatment of refractory chronic cough

Day 60 opinion

**Action:** For adoption

Pneumology - Allergology

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

Prevention of measles, mumps, rubella and varicella

Day 60 opinion

**Action:** For adoption

Vaccines / Infectious Diseases

#### 2.1.18. Varicella - EMEA-003317-PIP02-22

Prevention of varicella

Day 17 opinion

**Action:** For adoption

Vaccines / Infectious Diseases

### 2.1.19. Clascoterone - EMEA-003330-PIP01-22

---

Treatment of acne vulgaris

Day 60 opinion

**Action:** For adoption

Dermatology

## 2.2. Opinions on Compliance Check

### 2.2.1. *Neisseria meningitidis* serogroup B fHbp subfamily B / *Neisseria meningitidis* serogroup B fHbp subfamily A / *Neisseria meningitidis* group A polysaccharide conjugated to tetanus toxoid carrier protein / *Neisseria meningitidis* group W-135 polysaccharide conjugated to tetanus toxoid carrier protein / *Neisseria meningitidis* group Y polysaccharide conjugated to tetanus toxoid carrier protein / *Neisseria meningitidis* group C polysaccharide conjugated to tetanus toxoid carrier protein - EMEA-C1-002814-PIP02-21

---

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

Day 60 letter

**Action:** For adoption

Vaccines

### 2.2.1. Ivacaftor - EMEA-C-000335-PIP01-08-M15

---

Vertex Pharmaceuticals (Ireland) Limited; Treatment of cystic fibrosis

Day 30 opinion

**Action:** For adoption

Other

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

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

---

Neurocrine Therapeutics Ltd.; Treatment of congenital adrenal hyperplasia

Day 60 opinion

**Action:** For adoption

Endocrinology-Gynaecology-Fertility-Metabolism

### 2.3.2. Drospirenone / estetrol monohydrate - EMEA-001332-PIP01-12-M06

---

Estetra SRL; Prevention of pregnancy

Day 60 opinion

**Action:** For adoption

Endocrinology-Gynaecology-Fertility-Metabolism

### 2.3.3. Evinacumab - EMEA-002298-PIP01-17-M05

---

Ultragenyx Germany GmbH; Treatment of elevated cholesterol

Day 60 opinion

**Action:** For adoption

Endocrinology-Gynaecology-Fertility-Metabolism

### 2.3.4. Ibutamoren mesilate - Orphan - EMEA-003032-PIP01-21-M01

---

Lumos Pharma, Inc.; Treatment of growth hormone deficiency

Day 60 opinion

**Action:** For adoption

Endocrinology-Gynaecology-Fertility-Metabolism

### 2.3.5. Baricitinib - EMEA-001220-PIP01-11-M07

---

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

Day 60 opinion

**Action:** For adoption

Immunology-Rheumatology-Transplantation

### 2.3.6. Baloxavir marboxil - EMEA-002440-PIP01-18-M04

---

Roche Registration GmbH; Treatment of influenza / Prevention of influenza

Day 60 opinion

**Action:** For adoption

Infectious Diseases

### 2.3.7. Relebactam monohydrate / cilastatin sodium / imipenem monohydrate - EMEA-001809-PIP01-15-M03

---

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

Day 60 opinion

**Action:** For adoption

Infectious Diseases

### 2.3.8. Fremanezumab - EMEA-001877-PIP01-15-M03

---

Teva GmbH; Prevention of migraine headaches

Day 60 opinion

**Action:** For adoption

Neurology

*Note: Withdrawal request received on 11 January 2023*

### 2.3.9. Glycopyrronium bromide - EMEA-001366-PIP01-12-M03

---

Proveca Pharma Limited; Treatment of sialorrhoea

Day 60 opinion

**Action:** For adoption

Neurology

### 2.3.10. Quizartinib - Orphan - EMEA-001821-PIP01-15-M06

---

Daiichi Sankyo Europe GmbH; Treatment of acute myeloid leukaemia

Day 60 opinion

**Action:** For adoption

Oncology

### 2.3.11. Gilteritinib (as fumarate) - Orphan - EMEA-002064-PIP01-16-M04

---

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

Day 60 opinion

**Action:** For adoption

Oncology / Haematology-Hemostaseology

### 2.3.12. Inotuzumab ozogamicin - Orphan - EMEA-001429-PIP01-13-M07

---

Pfizer Europe MA EEIG; Treatment of acute lymphoblastic leukaemia

---

Day 60 opinion

**Action:** For adoption

Oncology / Haematology-Hemostaseology

---

### 2.3.13. Afamelanotide - Orphan - EMEA-000737-PIP02-11-M02

---

Clinuvel Europe Limited; Treatment of erythropoietic protoporphyria

Day 60 opinion

**Action:** For adoption

Other

---

### 2.3.14. Selexipag - EMEA-000997-PIP01-10-M06

---

Janssen-Cilag International NV; Treatment of pulmonary arterial hypertension

Day 60 opinion

**Action:** For adoption

Other

---

### 2.3.15. Begelomab - Orphan - EMEA-001744-PIP01-14-M02

---

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

Day 60 opinion

**Action:** For adoption

Other / Immunology-Rheumatology-Transplantation

---

### 2.3.16. Methoxflurane - EMEA-000334-PIP01-08-M11

---

Medical Developments UK Ltd; Treatment of acute pain

Day 60 opinion

**Action:** For adoption

Pain

---

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

---

AstraZeneca AB; Treatment of asthma

Day 60 opinion

**Action:** For adoption

Pneumology - Allergology

---



### 2.3.18. Cariprazine hydrochloride - EMEA-001652-PIP01-14-M04

---

Gedeon Richter Plc.; Treatment of schizophrenia

Day 60 opinion

**Action:** For adoption

Psychiatry

### 2.3.19. Lumasiran sodium - Orphan - EMEA-002079-PIP01-16-M03

---

Alnylam UK Limited; Treatment of primary hyperoxaluria type 1

Day 60 opinion

**Action:** For adoption

Uro-nephrology

### 2.3.20. Methylphenidate hydrochloride - EMEA-003189-PIP01-22-M01

---

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

Day 30 opinion

**Action:** For adoption

Neurology

### 2.3.21. Respiratory syncytial virus stabilised prefusion F subunit vaccine - EMEA-002795-PIP02-21-M01

---

Pfizer Europe MA EEIG; Prevention of lower respiratory tract disease caused by respiratory syncytial virus

Day 30 opinion

**Action:** For adoption

Vaccine

### 2.3.22. Autologous CD34+ hematopoietic stem cells with a CRISPR-edited erythroid enhancer region of the BCL11A gene - EMEA-002730-PIP04-21-M01

---

Vertex Pharmaceuticals (Ireland) Limited; Treatment of beta-thalassemia intermedia and major

D30 opinion

**Action:** For adoption

Haematology-Hemostasiology

#### 2.3.23. Risdiplam - Orphan - EMEA-002070-PIP01-16-M07

---

Roche Registration GmbH; Treatment of spinal muscular atrophy

Day 30 discussion

**Action:** For discussion

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. Tapentadol - EMEA-C1-000325-PIP01-08-M10

---

Grünenthal GmbH; Treatment of chronic pain

Day 30 letter

**Action:** For information

Pain

## 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. Pudexacianinium - EMEA-003099-PIP01-21

---

Visualisation of ureter

Day 90 discussion

---

**Action:** For discussion

Endocrinology-Gynaecology-Fertility-Metabolism / Diagnostic / Oncology /  
Gastroenterology-Hepatology / Uro-nephrology

### 3.1.2. [Cilgavimab / tixagevimab - EMEA-003079-PIP01-22](#)

---

Treatment of coronavirus disease 2019 (COVID-19) / Prevention of coronavirus disease 2019 (COVID-19)

Day 90 discussion

**Action:** For discussion

Infectious Diseases

### 3.1.3. [Vilobelimab - EMEA-003080-PIP03-22](#)

---

Treatment of severe coronavirus disease 2019 (COVID-19)

Day 90 discussion

**Action:** For discussion

Infectious Diseases

### 3.1.4. [Zilovertamab vedotin - Orphan - EMEA-003257-PIP01-22](#)

---

Merck Sharp & Dohme (Europe) Inc.; Treatment of all conditions included in the category of malignant neoplasms (except nervous system, haematopoietic, and lymphoid tissue) / Treatment of malignant neoplasms of haematopoietic and lymphoid tissue

Day 90 discussion

**Action:** For discussion

Oncology

### 3.1.5. [Recombinant human ectonucleotide pyrophosphatase/phosphodiesterase 1 fused to the Fc fragment of IgG1 - Orphan - EMEA-003232-PIP01-22](#)

---

Inozyme Pharma Ireland Limited; Treatment of ectonucleotide pyrophosphatase / phosphodiesterase 1 (ENPP1) deficiency

Day 90 discussion

**Action:** For discussion

Other / Endocrinology-Gynaecology-Fertility-Metabolism / Cardiovascular Diseases

### 3.1.6. [ABNCoV2 \(AV2-cVLP-RBD SARS-CoV-2\) - EMEA-003184-PIP01-22](#)

---

Prevention of coronavirus disease 2019 (COVID-19)

Day 90 discussion

**Action:** For discussion

Vaccines / Infectious Diseases

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

---

Calcilytix Therapeutics, Inc a BridgeBio Company; Treatment of hypoparathyroidism

Day 60 discussion

**Action:** For discussion

Endocrinology-Gynaecology-Fertility-Metabolism

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

---

Treatment of hypophosphatasia

Day 60 discussion

**Action:** For discussion

Endocrinology-Gynaecology-Fertility-Metabolism

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

---

Treatment of portal hypertension with compensated cirrhosis

Day 60 discussion

**Action:** For discussion

Gastroenterology-Hepatology

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

---

Treatment of hereditary angioedema

Day 60 discussion

**Action:** For discussion

Haematology-Hemostaseology

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

---

Treatment of systemic lupus erythematosus

Day 60 discussion

**Action:** For discussion

Immunology-Rheumatology-Transplantation

---

### 3.1.12. Trimodulin (human IgM, IgA, IgG solution) - EMEA-002883-PIP02-22

---

Treatment of coronavirus disease 2019 (COVID-19)

Day 60 discussion

**Action:** For discussion

Neonatology - Paediatric Intensive Care / Infectious Diseases

### 3.1.13. Recombinant human arylsulfatase A - Orphan - EMEA-002050-PIP02-22

---

Takeda Pharmaceuticals International AG Ireland Branch; Treatment of metachromatic leukodystrophy

Day 60 discussion

**Action:** For discussion

Neurology

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

---

Calico Life Sciences LLC; Treatment of vanishing white matter disease

Day 60 discussion

**Action:** For discussion

Neurology

### 3.1.15. Hemopexin, human - Orphan - EMEA-003333-PIP01-22

---

CSL Behring GmbH; Treatment of sickle cell disease

Day 60 discussion

**Action:** For discussion

Other

### 3.1.16. EMEA-003347-PIP01-22

---

Treatment of glomerulonephritis and nephrotic syndrome

Day 60 discussion

**Action:** For discussion

Uro-nephrology

3.1.17. [Single-stranded 5' capped mRNA encoding the HAs of the influenza virus - EMEA-003346-PIP01-22](#)

---

Prevention of influenza disease

Day 60 discussion

**Action:** For discussion

Vaccines

3.1.18. [Nizaracianine - EMEA-003367-PIP01-22](#)

---

Enhancement of the ureters during surgery

Day 30 discussion

**Action:** For discussion

Diagnostic

3.1.19. [Venglustat - Orphan - EMEA-001716-PIP07-22](#)

---

Genzyme Europe B.V.; Treatment of Gaucher disease

Day 30 discussion

**Action:** For discussion

Endocrinology-Gynaecology-Fertility-Metabolism

3.1.20. [Pyridoxal 5'-phosphate monohydrate - Orphan - EMEA-002404-PIP02-22](#)

---

Medicure Pharma Europe Limited; Treatment of pyridox(am)ine 5'-phosphate oxidase (PNPO) deficiency

Day 30 discussion

**Action:** For discussion

Endocrinology-Gynaecology-Fertility-Metabolism / Neurology

3.1.21. [Govorestat - Orphan - EMEA-003365-PIP01-22](#)

---

Applied Therapeutics, Inc; Treatment of galactosemia

Day 30 discussion

**Action:** For discussion

Endocrinology-Gynaecology-Fertility-Metabolism / Ophthalmology / Neurology

3.1.22. [Fazirsiran - Orphan - EMEA-003355-PIP01-22](#)

---

Takeda Pharma A/S; Treatment of alpha-1 antitrypsin deficiency-associated liver disease

---

Day 30 discussion

**Action:** For discussion

Gastroenterology-Hepatology

---

### 3.1.23. Inhibitor of receptor-interacting protein kinase 1 - EMEA-003356-PIP01-22

---

Treatment of ulcerative colitis

Day 30 discussion

**Action:** For discussion

Gastroenterology-Hepatology

---

### 3.1.24. Efzofitimod - EMEA-003352-PIP01-22

---

Treatment of sarcoidosis

Day 30 discussion

**Action:** For discussion

Immunology-Rheumatology-Transplantation

---

### 3.1.25. Ruzotolimod - EMEA-003363-PIP01-22

---

Treatment of chronic hepatitis B

Day 30 discussion

**Action:** For discussion

Infectious Diseases

---

### 3.1.26. Xalnesiran - EMEA-003362-PIP01-22

---

Treatment of chronic hepatitis B

Day 30 discussion

**Action:** For discussion

Infectious Diseases

---

### 3.1.27. Tozorakimab - EMEA-003360-PIP01-22

---

Treatment of acute respiratory failure

Day 30 discussion

**Action:** For discussion

Infectious Diseases / Pneumology - Allergology

### 3.1.28. Clonidine - EMEA-003198-PIP02-22

---

Treatment of attention deficit hyperactivity disorder

Day 30 discussion

**Action:** For discussion

Neurology

### 3.1.29. Utreloxastat - EMEA-003369-PIP01-22

---

Treatment of amyotrophic lateral sclerosis

Day 30 discussion

**Action:** For discussion

Neurology

### 3.1.30. EMEA-003271-PIP02-22

---

Treatment of primary generalised tonic-clonic seizures

Day 30 discussion

**Action:** For discussion

Neurology

### 3.1.31. EMEA-003358-PIP01-22

---

Treatment of breast malignant neoplasms

Day 30 discussion

**Action:** For discussion

Oncology

### 3.1.32. Lutetium-177 (177Lu) rhPSMA 10.1 - EMEA-003353-PIP01-22

---

Treatment of PSMA-expressing prostate cancer

Day 30 discussion

**Action:** For discussion

Oncology

### 3.1.33. Naxitamab - EMEA-002346-PIP02-22

---

Treatment of osteosarcoma

Day 30 discussion



**Action:** For discussion

Oncology

### 3.1.34. [Nemvaleukin alfa - EMEA-003357-PIP01-22](#)

---

Treatment of malignant neoplasms of the central nervous system / Treatment of all conditions included in the category of malignant neoplasms (except nervous system, haematopoietic and lymphoid tissue) / Treatment of solid malignant tumours (adults and children) / Treatment of malignant neoplasms of the lymphoid tissue / Treatment of malignant neoplasms of the lymphoid tissue (children)

Day 30 discussion

**Action:** For discussion

Oncology

### 3.1.35. [Tamibarotene - Orphan - EMEA-003329-PIP02-22](#)

---

Syros Pharmaceutical (Ireland) Limited; Treatment of RARA-positive patients with acute myeloid leukaemia (AML) / Treatment of RARA-positive patients with myelodysplastic syndromes (MDS)

Day 30 discussion

**Action:** For discussion

Oncology

### 3.1.36. [Trotabresib - EMEA-003361-PIP01-22](#)

---

Treatment of malignant neoplasms of the central nervous system

Day 30 discussion

**Action:** For discussion

Oncology

### 3.1.37. [Acoltremon - EMEA-003351-PIP01-22](#)

---

Treatment of dry eye disease

Day 30 discussion

**Action:** For discussion

Ophthalmology

### 3.1.38. [Ciclosporin - EMEA-003366-PIP01-22](#)

---

Treatment of dry eye disease (DED)

Day 30 discussion

**Action:** For discussion

Ophthalmology

### 3.1.39. Pabinafusp alfa - Orphan - EMEA-003033-PIP02-22

---

JCR Pharmaceuticals Co., Ltd.; Treatment of mucopolysaccharidosis II (Hunter 's syndrome)

Day 30 discussion

**Action:** For discussion

Other

### 3.1.40. Cannabidiol - Orphan - EMEA-003176-PIP02-22

---

Zynerba Pharmaceuticals Inc; Treatment of fragile X syndrome (FXS)

Day 30 discussion

**Action:** For discussion

Psychiatry

### 3.1.41. Inaxaplin - Orphan - EMEA-003368-PIP01-22

---

Vertex Pharmaceuticals (Ireland) Limited; Treatment of APOL1-mediated kidney disease

Day 30 discussion

**Action:** For discussion

Uro-nephrology

### 3.1.42. Meningococcal group Y oligosaccharide conjugated to *Corynebacterium diphtheriae* CRM197 protein / Meningococcal group W-135 oligosaccharide conjugated to *Corynebacterium diphtheriae* CRM197 protein / Meningococcal group C oligosaccharide conjugated to *Corynebacterium diphtheriae* CRM197 protein / Meningococcal group A oligosaccharide conjugated to *Corynebacterium diphtheriae* CRM197 protein - EMEA-000032-PIP02-22

---

Prevention of meningococcal meningitis

Day 30 discussion

**Action:** For discussion

Vaccines

### 3.1.43. Meningococcal group Y oligosaccharides conjugated to *Corynebacterium diphtheriae* CRM197 protein / Meningococcal group W-135 oligosaccharides conjugated to *Corynebacterium diphtheriae* CRM197 protein / Meningococcal group C oligosaccharides conjugated to *Corynebacterium diphtheriae* CRM197 protein / Meningococcal group A oligosaccharides conjugated to *Corynebacterium diphtheriae*

---

CRM197 protein / Outer membrane vesicles (OMV) from *N. meningitidis* / Recombinant *Neisseria meningitidis* group B fHbp 2-3-1.13NB fusion protein / Recombinant *Neisseria meningitidis* group B protein 961c / Recombinant *Neisseria meningitidis* group B protein 287-953 / Recombinant *Neisseria meningitidis* group B protein 936-741 - EMEA-003359-PIP01-22

---

Prevention of meningococcal disease

Day 30 discussion

**Action:** For discussion

Vaccines

### 3.1.44. Zapomeran - EMEA-003349-PIP01-22

---

Prevention of coronavirus disease 2019 (COVID-19)

Day 30 discussion

**Action:** For discussion

Vaccines

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

---

Treatment of vascular injuries

Day 60 discussion

**Action:** For discussion

Other

## 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. Beremagene geperpavec - EMEA-C-002472-PIP03-22

---

Krystal Biotech Netherlands B.V.; Treatment of dystrophic epidermolysis bullosa

Day 30 discussion

**Action:** For discussion

Dermatology

### 3.2.2. Alirocumab - EMEA-C-001169-PIP01-11-M05

---

sanofi-aventis recherche & développement; Treatment of elevated cholesterol

---

Day 30 discussion

**Action:** For discussion

Endocrinology-Gynaecology-Fertility-Metabolism

### 3.2.3. [Dasiglucagon - EMEA-C1-002233-PIP01-17-M01](#)

---

Zealand Pharma a/s; Treatment of hypoglycaemia

Day 30 discussion

**Action:** For discussion

Endocrinology-Gynaecology-Fertility-Metabolism

### 3.2.4. [Bezlotoxumab - EMEA-C-001645-PIP01-14-M04](#)

---

Merck Sharp & Dohme (Europe), Inc.; Prevention of recurrence of *Clostridioides difficile* infection

Day 30 discussion

**Action:** For discussion

Infectious Diseases

### 3.2.5. [Tenofovir \(disoproxil fumarate\) - EMEA-C-000533-PIP01-08-M11](#)

---

Gilead Sciences Intl Ltd; Treatment of chronic viral hepatitis B

Day 30 discussion

**Action:** For discussion

Infectious Diseases

### 3.2.6. [Satralizumab - EMEA-C2-001625-PIP01-14-M06](#)

---

Roche Registration GmbH; Treatment of neuromyelitis optica

Day 30 discussion

**Action:** For discussion

Neurology

### 3.2.7. [Fluticasone furoate / vilanterol - EMEA-C-000431-PIP01-08-M12](#)

---

Glaxo Group Limited; Treatment of asthma

Day 30 discussion

**Action:** For discussion

Pneumology - Allergology

### 3.2.8. Vortioxetine - EMEA-C-000455-PIP02-10-M09

---

H. Lundbeck A/S; Treatment of major depressive disorder

Day 30 discussion

**Action:** For discussion

Psychiatry

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

### 3.3.1. Remimazolam - EMEA-001880-PIP02-19-M04

---

PAION Deutschland GmbH; Sedation / General anaesthesia

Day 30 discussion

**Action:** For discussion

Anaesthesiology

### 3.3.2. Sotatercept - Orphan - EMEA-002756-PIP01-19-M02

---

Merck Sharp & Dohme B.V.; Treatment of pulmonary arterial hypertension

Day 30 discussion

**Action:** For discussion

Cardiovascular Diseases

### 3.3.3. Delgocitinib - EMEA-002329-PIP02-20-M02

---

LEO Pharma A/S; Treatment of chronic hand eczema

Day 30 discussion

**Action:** For discussion

Dermatology

### 3.3.4. Lebrikizumab - EMEA-002536-PIP01-18-M03

---

Eli Lilly and Company Limited; Treatment of atopic dermatitis

Day 30 discussion

**Action:** For discussion

Dermatology

### 3.3.5. Remibrutinib - EMEA-002582-PIP01-19-M02

---

Novartis Europharm Limited; Treatment of chronic spontaneous urticaria

Day 30 discussion

**Action:** For discussion

Dermatology

### 3.3.6. Denosumab - EMEA-000145-PIP02-12-M05

---

Amgen Europe B.V.; Treatment of osteoporosis

Day 30 discussion

**Action:** For discussion

Endocrinology-Gynaecology-Fertility-Metabolism

### 3.3.7. Glucagon analogue linked to a human immunoglobulin Fc fragment - Orphan - EMEA-003170-PIP01-21-M01

---

Hanmi Pharm. Co., Ltd.; Treatment of congenital hyperinsulinism

Day 30 discussion

**Action:** For discussion

Endocrinology-Gynaecology-Fertility-Metabolism

### 3.3.8. Hydroxypropyl- $\beta$ -cyclodextrin (HP $\beta$ CD) - Orphan - EMEA-002839-PIP01-20-M01

---

Cyclo Therapeutics Inc; Treatment of Niemann Pick disease type C

Day 30 discussion

**Action:** For discussion

Endocrinology-Gynaecology-Fertility-Metabolism

### 3.3.9. Osilodrostat - Orphan - EMEA-000315-PIP02-15-M03

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Recordati Rare Diseases SARL; Treatment of adrenal cortical hyperfunction

Day 30 discussion

**Action:** For discussion

Endocrinology-Gynaecology-Fertility-Metabolism

### 3.3.10. Obeticholic acid - Orphan - EMEA-001304-PIP02-13-M06

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Intercept Pharma International Ltd.; Treatment of primary biliary cholangitis (PBC)

Day 30 discussion

**Action:** For discussion

Gastroenterology-Hepatology

### 3.3.11. [Andexanet alfa - EMEA-001902-PIP01-15-M07](#)

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AstraZeneca AB; Prevention of factor Xa inhibitor associated haemorrhage / Treatment of factor Xa inhibitor associated haemorrhage

Day 30 discussion

**Action:** For discussion

Haematology-Hemostaseology

### 3.3.12. [Synthetic double-stranded siRNA oligonucleotide directed against antithrombin mRNA and covalently linked to a ligand containing three N-acetylgalactosamine residues - Orphan - EMEA-001855-PIP01-15-M04](#)

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Genzyme Europe B.V.; Treatment of haemophilia B / Treatment of haemophilia A

Day 30 discussion

**Action:** For discussion

Haematology-Hemostaseology

### 3.3.13. [Filgotinib - EMEA-001619-PIP04-17-M02](#)

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

Day 30 discussion

**Action:** For discussion

Immunology-Rheumatology-Transplantation

### 3.3.14. [Tofacitinib citrate - EMEA-000576-PIP01-09-M14](#)

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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

### 3.3.15. [Cefepime / enmetazobactam - EMEA-002240-PIP02-17-M02](#)

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Allegra Therapeutics GmbH; Treatment of infections due to aerobic gram-negative organisms in patients with limited treatment options / Treatment of urinary tract infections / Treatment of patients with bacteraemia that occurs in association with, or is suspected to be associated with cUTI or HAP or VAP infections / Hospital-acquired pneumonia (HAP),

including ventilator associated pneumonia (VAP)

Day 30 discussion

**Action:** For discussion

Infectious Diseases

### 3.3.16. Lamivudine / dolutegravir - EMEA-001940-PIP01-16-M05

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

Day 30 discussion

**Action:** For discussion

Infectious Diseases

### 3.3.17. Oritavancin (diphosphate) - EMEA-001270-PIP01-12-M06

Menarini International Operations Luxembourg S.A.; Treatment of acute bacterial skin and skin structure infections

Day 30 discussion

**Action:** For discussion

Infectious Diseases

### 3.3.18. Tedizolid phosphate - EMEA-001379-PIP01-12-M07

Merck Sharp & Dohme (Europe), Inc.; Treatment of acute bacterial skin and skin structure infections

Day 30 discussion

**Action:** For discussion

Infectious Diseases

### 3.3.19. Brivaracetam - EMEA-000332-PIP02-17-M04

UCB Pharma S.A.; Treatment of paediatric epilepsy syndromes

Day 30 discussion

**Action:** For discussion

Neurology

### 3.3.20. Eculizumab - Orphan - EMEA-000876-PIP03-14-M06

Alexion Europe SAS; Treatment of neuromyelitis optica spectrum disorders

Day 30 discussion

**Action:** For discussion



Neurology

### 3.3.21. Eladocagene exuparvovec - Orphan - EMEA-002435-PIP01-18-M03

PTC Therapeutic International Limited; Treatment of aromatic L-amino acid decarboxylase deficiency

Day 30 discussion

**Action:** For discussion

Neurology

### 3.3.22. Rimegepant - EMEA-002812-PIP02-20-M02

Biohaven Pharmaceutical Ireland DAC; Treatment of migraine headaches

Day 30 discussion

**Action:** For discussion

Neurology

### 3.3.23. Satralizumab - Orphan - EMEA-001625-PIP02-21-M01

Roche Registration GmbH; Treatment of myasthenia gravis

Day 30 discussion

**Action:** For discussion

Neurology

### 3.3.24. Acalabrutinib - Orphan - EMEA-001796-PIP03-16-M03

Acerta Pharma, BV; Treatment of mature B cell neoplasms

Day 30 discussion

**Action:** For discussion

Oncology

### 3.3.25. Carfilzomib - Orphan - EMEA-001806-PIP04-19-M01

Amgen Europe BV; Treatment of acute lymphoblastic leukaemia

Day 30 discussion

**Action:** For discussion

Oncology

### 3.3.26. Selpercatinib - EMEA-002544-PIP01-18-M02

Eli Lilly and Company; Treatment of all conditions included in the category of malignant

neoplasms (except haematopoietic and lymphoid tissue neoplasms)

Day 30 discussion

**Action:** For discussion

Oncology

### 3.3.27. Temozolomide - EMEA-002634-PIP01-19-M02

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Accord Healthcare S.L.U.; Treatment of malignant glioma, such as glioblastoma multiforme or anaplastic astrocytoma

Day 30 discussion

**Action:** For discussion

Oncology

### 3.3.28. Botaretigene sparoparovec - Orphan - EMEA-002827-PIP01-20-M01

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Janssen-Cilag International NV; Treatment of retinitis pigmentosa

Day 30 discussion

**Action:** For discussion

Ophthalmology

### 3.3.29. Patiromer sorbitex calcium - EMEA-001720-PIP01-14-M03

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Vifor Fresenius Medical Care Renal Pharma France; Treatment of hyperkalaemia

Day 30 discussion

**Action:** For discussion

Other

### 3.3.30. Zilucoplan - Orphan - EMEA-002747-PIP01-20-M01

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UCB Pharma SA; Treatment of myasthenia gravis

Day 30 discussion

**Action:** For discussion

Other / Neurology

### 3.3.31. Defatted powder of peanuts - EMEA-001734-PIP01-14-M06

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Aimmune Therapeutics Inc; Treatment of peanut allergy

Day 30 discussion

**Action:** For discussion

Pneumology - Allergology

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### 3.3.32. Ravulizumab (ALXN1210) - EMEA-001943-PIP02-20-M02

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Alexion Europe SAS; Treatment of patients with haematopoietic stem cell transplant associated thrombotic microangiopathy (HSCT-TMA)

Day 30 discussion

**Action:** For discussion

Uro-nephrology / Haematology-Hemostaseology

## 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 30 January 2023 for Nomination of Rapporteur and Peer reviewer

**Action:** For adoption

### 4.2. Nomination of Rapporteur for requests of confirmation on the applicability of the EMA decision on class waiver

**Action:** For adoption

### 4.3. Nominations for other activities

**Action:** For adoption

## 5. Scientific Advice Working Party (SAWP) and Paediatric Committee (PDCO) Interaction

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

### 5.1.1. Committee representatives at SAWP: call for re-nomination

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**Action:** For discussion

## 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. EMEA-08-2022

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AbbVie Ltd; All classes of medicinal products for treatment of Alzheimer's disease;  
Treatment of dementia of the Alzheimer's type

**Action:** For adoption

### 6.1.2. Evorpacept - EMEA-10-2022

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ALX Oncology, Inc; The class of Her- / epidermal growth factor-receptor antibody medicinal products for treatment of intestinal malignant neoplasms; The class of pyrimidine- and pyrimidine analogue-containing medicinal products AND the class of first- and second-generation platinum-containing medicinal products the for treatment of head and neck epithelial malignant neoplasms

**Action:** For adoption

### 6.1.3. Lonsurf - EMEA-04-2022

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Les Laboratoires Servier; The class of pyrimidine- and pyrimidine analogue-containing medicinal products for treatment of breast malignant neoplasms, intestinal malignant neoplasms, lung malignant neoplasms, pancreatic malignant neoplasms, head and neck epithelial malignant neoplasms, skin malignant neoplasms and actinic keratosis; Lonsurf is indicated in combination with bevacizumab for the treatment of adult patients with metastatic colorectal cancer (CRC) who have received two prior anticancer treatment regimens

**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

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**Action:** For information

#### 9.1.2. Vote by Proxy

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**Action:** For information

#### 9.1.3. Strategic Review and Learning Meeting (SRLM) - Stockholm, 7-8 June 2023

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PDCO member: Kristin Karlsson

**Action:** For information

### 9.2. Coordination with EMA Scientific Committees or CMDh-v

#### 9.2.1. Committee for Medicinal Products for Human Use (CHMP)

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**Action:** For information

### 9.3. Coordination with EMA Working Parties/Working Groups/Drafting Groups

#### 9.3.1. Non-clinical Working Party: D30 Products identified

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PDCO member: Karen van Malderen

**Action:** For information

#### 9.3.2. Formulation Working Group

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PDCO member: Brian Aylward (*ad interim*)

**Action:** For information

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

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No item

## 9.4. Cooperation within the EU regulatory network

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

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**Action:** For information

## 9.5. Cooperation with International Regulators

### 9.5.1. Paediatric Cluster Teleconference

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**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

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No item

## 10. Any other business

### 10.1. Regulatory and scientific virtual conference on RNA based medicines

**Action:** For information

### 10.2. COVID-19 update

**Action:** For information

### 10.3. Report on the conclusions of the Workshop on 'Toward Consensus on Best Practices for the Design and Conduct of Pediatric Obesity Pharmacotherapy Clinical Trials'

PDCO member: Helena Fonseca

**Action:** For information

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#### **10.4. Revision of the guideline on clinical investigation of medicinal products in the treatment or prevention of diabetes mellitus**

PDCO member: Carine de Beaufort

**Action:** For discussion

#### **10.5. Upcoming Innovation Task Force (ITF) meetings**

**Action:** For information

#### **10.6. Update on the EMA funded study on spinal muscular atrophy**

**Action:** For information

### **11. Breakout sessions**

#### **11.1. Internal PDCO Operations**

**Action:** For discussion on Tuesday, 11:00 - 12:00

#### **11.2. HIV**

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

#### **11.3. Paediatric oncology**

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

#### **11.4. Neonatology**

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

#### **11.5. Vaccines**

**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.

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