



EUROPEAN MEDICINES AGENCY  
SCIENCE MEDICINES HEALTH

15 June 2023  
EMA/PDCO/242901/2023  
Human Medicines Division

## Paediatric Committee (PDCO)

Draft Agenda for the meeting on 20-23 June 2023

Chair: Brian Aylward – Vice-Chair: Sylvie Benchetrit

20 June 2023, 14:00 - 19:45, room 2C

21 June 2023, 08:30 - 19:45, room 2C

22 June 2023, 08:30 - 19:45, room 2C

23 June 2023, 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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## 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 20-23 June 2023. See June 2023 PDCO minutes (to be published post July 2023 PDCO meeting).

### 1.2. Adoption of agenda

PDCO agenda for 20-23 June 2023.

### 1.3. Adoption of the minutes

PDCO minutes for 23-26 May 2023.

## 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. Furosemide - EMEA-003316-PIP01-22

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Treatment of fluid retention

Day 120 opinion

**Action:** For adoption

Cardiovascular Diseases

#### 2.1.2. Milvexian - EMEA-003220-PIP01-22

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Prevention of thromboembolism

Day 120 opinion

**Action:** For adoption

Cardiovascular Diseases

#### 2.1.3. EMEA-003301-PIP01-22

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Treatment of psoriasis

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Day 120 opinion

**Action:** For adoption

Dermatology

#### [2.1.4. Povorcitinib - EMEA-003313-PIP01-22](#)

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Treatment of hidradenitis suppurativa

Day 120 opinion

**Action:** For adoption

Dermatology

#### [2.1.5. Ritlecitinib - EMEA-002451-PIP03-22](#)

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Treatment of vitiligo

Day 120 opinion

**Action:** For adoption

Dermatology

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

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Calcilytix Therapeutics, Inc a BridgeBio Company; Treatment of hypoparathyroidism

Day 120 opinion

**Action:** For adoption

Endocrinology-Gynaecology-Fertility-Metabolism

#### [2.1.7. Dirloctocogene samoparvovec - Orphan - EMEA-003290-PIP01-22](#)

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Spark Therapeutics Ireland Limited; Treatment of haemophilia A

Day 120 opinion

**Action:** For adoption

Haematology-Hemostaseology

#### [2.1.8. Mocravimod - Orphan - EMEA-003304-PIP01-22](#)

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Priothera SAS; Treatment in haematopoietic stem cell transplantation (HSCT)

Day 120 opinion

**Action:** For adoption

Haematology-Hemostaseology

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

---

Takeda Pharmaceuticals International AG Ireland Branch; Treatment of metachromatic leukodystrophy

Day 120 opinion

**Action:** For adoption

Neurology

*Note: Withdrawal request received on 12 June 2023*

### 2.1.10. EMEA-003271-PIP01-22

---

Treatment of focal onset seizures

Day 120 opinion

**Action:** For adoption

Neurology

### 2.1.11. EMEA-003274-PIP01-22

---

Treatment of melanoma

Day 120 opinion

**Action:** For adoption

Oncology

### 2.1.12. Dersimelagon - EMEA-002850-PIP03-22

---

Treatment of systemic sclerosis

Day 120 opinion

**Action:** For adoption

Other

### 2.1.13. mRNA encoding modified human ornithine transcarbamylase - Orphan - EMEA-003315-PIP01-22

---

Arcturus Therapeutics Europe B.V.; Treatment of ornithine transcarbamylase deficiency

Day 120 opinion

**Action:** For adoption

Other

#### 2.1.14. Pamrevlumab - EMEA-002979-PIP04-22

---

Treatment of interstitial lung diseases with fibrosis

Day 120 opinion

**Action:** For adoption

Pneumology - Allergology

#### 2.1.15. Repagermanium - Orphan - EMEA-003154-PIP01-21

---

Dimerix Bioscience Pty Ltd; Treatment of focal segmental glomerulosclerosis (FSGS)

Day 120 opinion

**Action:** For adoption

Uro-nephrology

#### 2.1.16. Influenza recombinant H7 haemagglutinin - EMEA-003314-PIP01-22

---

Prevention of influenza infection

Day 120 opinion

**Action:** For adoption

Vaccines

#### 2.1.17. Single-stranded 5' capped mRNA encoding the HAs of the influenza virus - EMEA-003346-PIP01-22

---

Prevention of influenza disease

Day 120 opinion

**Action:** For adoption

Vaccines

#### 2.1.18. MVA-BN-RSV - EMEA-003185-PIP01-22

---

Prevention of lower respiratory tract disease (LRTD) caused by respiratory syncytial virus (RSV)

Day 120 opinion

**Action:** For adoption

Vaccines / Infectious Diseases

#### 2.1.19. Semaglutide - EMEA-001441-PIP08-23

---

Prevention of cardiovascular events in patients with atherosclerosis

---

Day 60 opinion

**Action:** For adoption

Endocrinology-Gynaecology-Fertility-Metabolism

---

#### 2.1.20. [Semaglutide / cagrilintide - EMEA-003059-PIP02-23](#)

---

Treatment of type 2 diabetes mellitus

Day 60 opinion

**Action:** For adoption

Endocrinology-Gynaecology-Fertility-Metabolism

---

#### 2.1.21. [Rilzabrutinib - Orphan - EMEA-002438-PIP04-23](#)

---

Sanofi B.V.; Treatment of warm autoimmune haemolytic anaemia

Day 60 opinion

**Action:** For adoption

Haematology-Hemostaseology

---

#### 2.1.22. [Sotuletinib - Orphan - EMEA-003415-PIP01-23](#)

---

Novartis Europharm Limited; Treatment of amyotrophic lateral sclerosis (ALS)

Day 60 opinion

**Action:** For adoption

Neurology

---

#### 2.1.23. [Atezolizumab / tiragolumab - EMEA-003418-PIP01-23](#)

---

Treatment of lung carcinoma (non-small cell carcinoma)

Day 60 opinion

**Action:** For adoption

Oncology

---

#### 2.1.24. [Autologous dendritic cells pulsed with allogeneic tumour cell lysate - Orphan - EMEA-002381-PIP02-23](#)

---

Amphera BV; Treatment of pancreatic cancer

Day 60 opinion

**Action:** For adoption

Oncology

---

#### 2.1.25. Domvanalimab - EMEA-003429-PIP01-23

---

Treatment of lung cancer

Day 60 opinion

**Action:** For adoption

Oncology

#### 2.1.26. Domvanalimab - EMEA-003429-PIP02-23

---

Treatment of gastric, gastroesophageal junction, and oesophageal adenocarcinoma

Day 60 opinion

**Action:** For adoption

Oncology

#### 2.1.27. Tazemetostat - Orphan - EMEA-003055-PIP02-23

---

Ipsen Pharma; Treatment of follicular lymphoma

Day 60 opinion

**Action:** For adoption

Oncology

#### 2.1.28. Zimberelimab - EMEA-003427-PIP01-23

---

Treatment of lung cancer

Day 60 opinion

**Action:** For adoption

Oncology

#### 2.1.29. Zimberelimab - EMEA-003427-PIP02-23

---

Treatment of gastric, gastroesophageal junction, and oesophageal adenocarcinoma

Day 60 opinion

**Action:** For adoption

Oncology

#### 2.1.30. Satralizumab - Orphan - EMEA-001625-PIP05-23

---

Roche Registration GmbH; Treatment of thyroid eye disease (TED)

Day 60 opinion

**Action:** For adoption

Ophthalmology / Neurology

---

2.1.31. [Sodium lactate / xylitol / magnesium chloride hexahydrate / calcium chloride dihydrate / sodium chloride / L-carnitine / D-glucose - EMEA-003417-PIP01-23](#)

---

Treatment of patients in need of peritoneal dialysis

Day 60 opinion

**Action:** For adoption

Uro-nephrology

*Note: Withdrawal request received on 12 June 2023*

## 2.2. **Opinions on Compliance Check**

---

2.2.1. [Clascoterone - EMEA-C-003330-PIP01-22](#)

---

Cassiopea S.p.A; Treatment of acne vulgaris

Day 60 opinion

**Action:** For adoption

Dermatology

---

2.2.2. [Marstacimab - EMEA-C1-002285-PIP02-19-M02](#)

---

Pfizer Europe MA EEIG; Treatment of congenital haemophilia A

Day 60 letter

**Action:** For adoption

Haematology-Hemostaseology

---

2.2.3. [Rilpivirine \(hydrochloride\) - EMEA-C-000317-PIP01-08-M13](#)

---

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

Day 60 opinion

**Action:** For adoption

Infectious Diseases

---

2.2.4. [Teriflunomide - EMEA-C-001094-PIP01-10-M04](#)

---

Sanofi Aventis Groupe; Treatment of multiple sclerosis

Day 60 opinion

**Action:** For adoption

Neurology

#### 2.2.5. [Imetelstat - EMEA-C1-001910-PIP03-20-M01](#)

---

Geron Corporation; Treatment of myelodysplastic syndromes (MDS), including juvenile myelomonocytic leukaemia (JMML)

Day 60 letter

**Action:** For adoption

Oncology

#### 2.2.6. [Peanut \(\*Arachis hypogaea\*\) allergen powder \(previously known as peanut flour\) - EMEA-C-001734-PIP01-14-M06](#)

---

Aimmune Therapeutics Ireland Ltd; Treatment of peanut allergy

Day 60 opinion

**Action:** For adoption

Pneumology - Allergology

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

#### 2.3.1. [Beremagene geperpavec - Orphan - EMEA-002472-PIP03-22-M01](#)

---

Krystal Biotech, Inc.; Treatment of dystrophic epidermolysis bullosa

Day 60 opinion

**Action:** For adoption

Dermatology

#### 2.3.2. [Dupilumab - EMEA-001501-PIP07-20-M01](#)

---

Sanofi Winthrop Industrie; Treatment of chronic spontaneous urticaria

Day 60 opinion

**Action:** For adoption

Dermatology

#### 2.3.3. [Inclisiran - EMEA-002214-PIP01-17-M02](#)

---

Novartis Europharm Ltd.; Treatment of elevated cholesterol



Day 60 opinion

**Action:** For adoption

Endocrinology-Gynaecology-Fertility-Metabolism

---

#### 2.3.4. Obeticholic acid - Orphan - EMEA-001304-PIP02-13-M07

---

Advanz Pharma Limited; Treatment of primary biliary cholangitis (PBC)

Day 60 opinion

**Action:** For adoption

Gastroenterology-Hepatology

---

#### 2.3.5. PF-06865571 - EMEA-002773-PIP01-20-M01

---

Pfizer Europe MA EEIG; Treatment of non-alcoholic steatohepatitis (NASH)

Day 60 opinion

**Action:** For adoption

Gastroenterology-Hepatology

---

#### 2.3.6. Iron as ferric maltol - EMEA-001195-PIP01-11-M06

---

Norgine BV; Treatment of iron deficiency anaemia (IDA)

Day 60 opinion

**Action:** For adoption

Haematology-Hemostaseology

---

#### 2.3.7. Ibrutinib - EMEA-001397-PIP04-17-M02

---

Janssen-Cilag International NV; Treatment of chronic graft-versus-host disease

Day 60 opinion

**Action:** For adoption

Immunology-Rheumatology-Transplantation

---

#### 2.3.8. Tofacitinib citrate - EMEA-000576-PIP01-09-M15

---

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

---

### 2.3.9. Delandistrogene moxeparovec - Orphan - EMEA-002677-PIP01-19-M03

Roche Registration GmbH; Treatment of Duchenne muscular dystrophy

Day 60 opinion

**Action:** For adoption

Neurology

### 2.3.10. Erenumab - EMEA-001664-PIP02-15-M06

Novartis Europharm Limited; Prevention of migraine headaches

Day 60 opinion

**Action:** For adoption

Neurology

### 2.3.11. Lacosamide - EMEA-000402-PIP03-17-M07

UCB Pharma S.A.; Treatment of generalised epilepsy and epilepsy syndromes

Day 60 opinion

**Action:** For adoption

Neurology

### 2.3.12. Abemaciclib - EMEA-002342-PIP01-18-M03

Eli Lilly and Company Limited; Treatment of Ewing's sarcoma

Day 60 opinion

**Action:** For adoption

Oncology

### 2.3.13. Abemaciclib - EMEA-002342-PIP02-18-M02

Eli Lilly and Company Limited; Treatment of glioma / Treatment of neuroblastoma

Day 60 opinion

**Action:** For adoption

Oncology

### 2.3.14. Avapritinib - Orphan - EMEA-002358-PIP02-18-M03

Blueprint Medicines (Netherlands) B.V.; Treatment of all conditions included in the category of malignant neoplasms (except haematopoietic and lymphoid tissue neoplasms)

Day 60 opinion

**Action:** For adoption

Oncology

---

#### 2.3.15. Cabozantinib - Orphan - EMEA-001143-PIP01-11-M06

---

Ipsen Pharma; Treatment of malignant solid tumours

Day 60 opinion

**Action:** For adoption

Oncology

---

#### 2.3.16. Olaparib - EMEA-002269-PIP01-17-M02

---

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

Day 60 opinion

**Action:** For adoption

Oncology

---

#### 2.3.17. Quizartinib - Orphan - EMEA-001821-PIP01-15-M07

---

Daiichi Sankyo Europe GmbH; Treatment of acute myeloid leukaemia

Day 60 opinion

**Action:** For adoption

Oncology

---

#### 2.3.18. Bupropion (hydrochloride) / naltrexone (hydrochloride) - EMEA-001373-PIP01-12-M05

---

Orexigen Therapeutics Ireland Limited; Treatment of obesity

Day 60 opinion

**Action:** For adoption

Other

---

#### 2.3.19. Tirzepatide - EMEA-002360-PIP02-22-M01

---

Eli Lilly and Company; Treatment of obesity

Day 60 opinion

**Action:** For adoption

---

Other

### 2.3.20. Ketamine / sufentanil - EMEA-001739-PIP02-16-M02

---

Cessatech A/S; Treatment of acute pain

Day 60 opinion

**Action:** For adoption

Pain

### 2.3.21. Durvalumab - EMEA-002028-PIP01-16-M04

---

AstraZeneca AB; Treatment of all conditions included in the category of malignant neoplasms (except central nervous system, haematopoietic and lymphoid tissue)

Day 30 opinion

**Action:** For adoption

Oncology

### 2.3.22. Tremelimumab - EMEA-002029-PIP01-16-M04

---

AstraZeneca AB; Treatment of all conditions included in the category of malignant neoplasms (except central nervous system, haematopoietic and lymphoid tissue)

Day 30 opinion

**Action:** For adoption

Oncology

## 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. Alprazolam - EMEA-C1-003043-PIP01-21

---

UCB Pharma SA; Treatment of epileptic seizures

Day 30 letter

**Action:** For information

Neurology

## 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. Dexmedetomidine - EMEA-003283-PIP01-22

---

Sedation

Day 90 discussion

**Action:** For discussion

Anaesthesiology

#### 3.1.2. Barzolvolimab - EMEA-003327-PIP01-22

---

Treatment of chronic spontaneous urticaria

Day 90 discussion

**Action:** For discussion

Dermatology

#### 3.1.3. Isotretinoin - Orphan - EMEA-003303-PIP01-22

---

Timber Pharmaceuticals LLC; Treatment of congenital ichthyosis

Day 90 discussion

**Action:** For discussion

Dermatology

#### 3.1.4. Upadacitinib - EMEA-001741-PIP07-22

---

Treatment of vitiligo

Day 90 discussion

**Action:** For discussion

Dermatology

---

3.1.5. [Humanised monoclonal antibody derivative against fibroblast growth factor receptor 3 - Orphan - EMEA-003253-PIP01-22](#)

---

Sanofi B.V.; Treatment of achondroplasia

Day 90 discussion

**Action:** For discussion

Endocrinology-Gynaecology-Fertility-Metabolism

---

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

---

Treatment of hypophosphatasia

Day 90 discussion

**Action:** For discussion

Endocrinology-Gynaecology-Fertility-Metabolism

---

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

---

Applied Therapeutics, Inc; Treatment of galactosaemia

Day 90 discussion

**Action:** For discussion

Endocrinology-Gynaecology-Fertility-Metabolism / Ophthalmology / Neurology

---

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

---

Treatment of portal hypertension

Day 90 discussion

**Action:** For discussion

Gastroenterology-Hepatology

---

3.1.9. [Obinutuzumab - Orphan - EMEA-001207-PIP05-22](#)

---

Roche Registration GmbH; Treatment of glomerulonephritis and nephrotic syndrome

Day 90 discussion

**Action:** For discussion

Immunology-Rheumatology-Transplantation

### 3.1.10. EMEA-003326-PIP01-22

---

Treatment of infections caused by gram-negative organisms / Complicated urinary tract infections (cUTI) / Hospital associated pneumonia or ventilator associated pneumonia

Day 90 discussion

**Action:** For discussion

Infectious Diseases

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

---

Treatment of coronavirus disease 2019 (COVID-19)

Day 90 discussion

**Action:** For discussion

Neonatology - Paediatric Intensive Care / Infectious Diseases

### 3.1.12. 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 90 discussion

**Action:** For discussion

Neurology

### 3.1.13. Vesleteplirsen - EMEA-003305-PIP01-22

---

Treatment of Duchenne muscular dystrophy

Day 90 discussion

**Action:** For discussion

Neurology

### 3.1.14. Retatrutide - EMEA-003258-PIP01-22

---

Treatment of obesity

Day 90 discussion

**Action:** For discussion

Other

3.1.15. 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 90 discussion

**Action:** For discussion

Other

3.1.16. Dexpramipexole - EMEA-003328-PIP01-22

---

Treatment of asthma

Day 90 discussion

**Action:** For discussion

Pneumology - Allergology

3.1.17. EMEA-003319-PIP02-22

---

Treatment of major depressive disorder (MDD)

Day 90 discussion

**Action:** For discussion

Psychiatry

3.1.18. EMEA-003319-PIP03-22

---

Treatment of post-traumatic stress disorder (PTSD)

Day 90 discussion

**Action:** For discussion

Psychiatry

3.1.19. Remibrutinib - EMEA-002582-PIP03-23

---

Treatment chronic inducible urticaria

Day 60 discussion

**Action:** For discussion

Dermatology

3.1.20. Spesolimab - EMEA-002475-PIP04-23

---

Treatment of hidradenitis suppurativa

---



Day 60 discussion

**Action:** For discussion

Dermatology

### 3.1.21. [EMEA-003414-PIP01-23](#)

---

Treatment of type 2 diabetes mellitus

Day 60 discussion

**Action:** For discussion

Endocrinology-Gynaecology-Fertility-Metabolism

### 3.1.22. [EMEA-003414-PIP02-23](#)

---

Treatment of obesity

Day 60 discussion

**Action:** For discussion

Endocrinology-Gynaecology-Fertility-Metabolism

### 3.1.23. [Frexalimab - EMEA-002945-PIP03-23](#)

---

Treatment of type 1 diabetes mellitus

Day 60 discussion

**Action:** For discussion

Endocrinology-Gynaecology-Fertility-Metabolism

### 3.1.24. [Mavodelpar - Orphan - EMEA-003331-PIP02-23](#)

---

Reneo Pharmaceuticals Inc; Treatment of primary mitochondrial disorders

Day 60 discussion

**Action:** For discussion

Endocrinology-Gynaecology-Fertility-Metabolism

### 3.1.25. [Modified messenger ribonucleic acid encoding human propionyl-coenzyme A carboxylase alpha and beta subunits encapsulated into lipid nanoparticles - Orphan - EMEA-003419-PIP01-23](#)

---

Moderna Biotech Spain, S.L.; Treatment of propionic acidaemia

Day 60 discussion

**Action:** For discussion

Endocrinology-Gynaecology-Fertility-Metabolism

### [3.1.26. Sepiapterin - Orphan - EMEA-003027-PIP02-23](#)

---

PTC Therapeutics International; Treatment of hyperphenylalaninaemia

Day 60 discussion

**Action:** For discussion

Endocrinology-Gynaecology-Fertility-Metabolism

### [3.1.27. Cobitolimod - Orphan - EMEA-001589-PIP02-23](#)

---

InDex Pharmaceuticals AB; Treatment of ulcerative colitis

Day 60 discussion

**Action:** For discussion

Gastroenterology-Hepatology

### [3.1.28. Autologous CD34+ cells transduced with a lentiviral vector containing a codon-optimised RPS19 gene - Orphan - EMEA-003074-PIP02-23](#)

---

Apriligen LLC; Treatment of Diamond-Blackfan anaemia

Day 60 discussion

**Action:** For discussion

Haematology-Hemostaseology

### [3.1.29. Belumosudil - Orphan - EMEA-003425-PIP01-23](#)

---

Sanofi Winthrop Industrie; Treatment of transplant complications

Day 60 discussion

**Action:** For discussion

Immunology-Rheumatology-Transplantation

### [3.1.30. Trimodulin \(human IgM, IgA, IgG solution\) - EMEA-002883-PIP03-23](#)

---

Treatment of lower respiratory tract and lung infections

Day 60 discussion

**Action:** For discussion

Neonatology - Paediatric Intensive Care / Infectious Diseases / Pneumology - Allergology

### 3.1.31. Abemaciclib - EMEA-002342-PIP04-23

---

Treatment of neuroblastoma

Day 60 discussion

**Action:** For discussion

Oncology

### 3.1.32. Blinatumomab - Orphan - EMEA-000574-PIP03-23

---

Amgen Europe B.V.; Treatment of acute lymphoblastic leukaemia

Day 60 discussion

**Action:** For discussion

Oncology

### 3.1.33. Olutasidenib - Orphan - EMEA-003421-PIP01-23

---

Rigel Pharmaceuticals B.V.; Treatment of acute myeloid leukaemia

Day 60 discussion

**Action:** For discussion

Oncology

### 3.1.34. Volrustomig - EMEA-003423-PIP01-23

---

Treatment of lung cancer (small cell and non-small cell lung cancer) / Treatment of renal cell cancer / Treatment of cervical cancer / Treatment of malignant pleural mesothelioma

Day 60 discussion

**Action:** For discussion

Oncology

### 3.1.35. Cedazuridine / decitabine - Orphan - EMEA-003071-PIP02-23

---

Otsuka Pharmaceutical Netherlands B.V.; Treatment of myelodysplastic syndromes

Day 60 discussion

**Action:** For discussion

Oncology / Haematology-Hemostaseology

### 3.1.36. Iptacopan - EMEA-002705-PIP05-23

---

Treatment of immune-complex mediated membranoproliferative glomerulonephritis (IC-MPGN)

---

Day 60 discussion

**Action:** For discussion

Other

**3.1.37. Autologous cell product composed of CD34+ enriched haematopoietic stem cells (HSCs) that have been genetically modified to express the functional  $\beta$ -glucocerebrosidase (GCase) protein - Orphan - EMEA-003424-PIP01-23**

---

AVROBIO, Inc.; Treatment of Gaucher disease type 1 / Treatment of Gaucher disease type 2 / Treatment of Gaucher disease type 3

Day 60 discussion

**Action:** For discussion

Other / Pain / Endocrinology-Gynaecology-Fertility-Metabolism / Pneumology - Allergology / Gastroenterology-Hepatology / Haematology-Hemostaseology / Neurology

**3.1.38. mRNA encoding for the linked NTD and RBD domains of the spike glycoprotein of SARS-CoV-2 - EMEA-003426-PIP01-23**

---

Prevention of coronavirus disease 2019 (COVID-19)

Day 60 discussion

**Action:** For discussion

Vaccines / Infectious Diseases

**3.1.39. Obicetrapib - EMEA-003438-PIP01-23**

---

Treatment of mixed dyslipidaemia

Day 30 discussion

**Action:** For discussion

Cardiovascular Diseases

**3.1.40. Obicetrapib - EMEA-003438-PIP02-23**

---

Treatment of elevated cholesterol

Day 30 discussion

**Action:** For discussion

Cardiovascular Diseases

**3.1.41. Ramipril / nebivolol - EMEA-003430-PIP01-23**

---

Treatment of hypertension

Day 30 discussion

**Action:** For discussion

Cardiovascular Diseases

---

3.1.42. [Modified mRNA encoding human methylmalonyl-coenzyme A mutase containing a polymorphism at position 671 - Orphan - EMEA-003437-PIP01-23](#)

---

Moderna Biotech Spain, S.L.; Treatment of methylmalonic acidaemia

Day 30 discussion

**Action:** For discussion

Endocrinology-Gynaecology-Fertility-Metabolism

---

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

---

Ascendis Pharma Growth Disorders A/S; Treatment of achondroplasia

Day 30 discussion

**Action:** For discussion

Endocrinology-Gynaecology-Fertility-Metabolism

---

3.1.44. [Synthetic double-stranded siRNA oligonucleotide directed against apolipoprotein C-III mRNA and covalently linked to a ligand containing three N-acetylgalactosamine residues - Orphan - EMEA-003420-PIP01-23](#)

---

Arrowhead Pharmaceuticals, Inc.; Treatment of familial chylomicronemia syndrome (FCS)

Day 30 discussion

**Action:** For discussion

Endocrinology-Gynaecology-Fertility-Metabolism

---

3.1.45. [Dupilumab - EMEA-001501-PIP12-23](#)

---

Treatment of eosinophilic gastroenteritis

Day 30 discussion

**Action:** For discussion

Gastroenterology-Hepatology

---

3.1.46. [Pemafibrate - EMEA-001573-PIP03-23](#)

---

Treatment of primary biliary cholangitis

Day 30 discussion

**Action:** For discussion

---

Gastroenterology-Hepatology

### 3.1.47. Tarperprumig - EMEA-003432-PIP01-23

Treatment of patients with sickle cell disease (SCD)

Day 30 discussion

**Action:** For discussion

Haematology-Hemostaseology

### 3.1.48. Amiodarone - EMEA-003428-PIP01-23

Treatment of supraventricular tachyarrhythmias in children from birth to less than 18 years of age

Day 30 discussion

**Action:** For discussion

Neonatology - Paediatric Intensive Care / Cardiovascular Diseases

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

Marinus Pharmaceuticals Inc.; Treatment of tuberous sclerosis complex

Day 30 discussion

**Action:** For discussion

Neurology

### 3.1.50. Tralesinidase alfa - Orphan - EMEA-003433-PIP01-23

Labcorp Clinical Development Limited; Treatment of mucopolysaccharidosis type III B (Sanfilippo B syndrome)

Day 30 discussion

**Action:** For discussion

Neurology

### 3.1.51. Abemaciclib - EMEA-002342-PIP05-23

Treatment of prostate cancer

Day 30 discussion

**Action:** For discussion

Oncology

### 3.1.52. Binimetinib - EMEA-001454-PIP06-23

---

Treatment of non-small cell lung cancer

Day 30 discussion

**Action:** For discussion

Oncology

### 3.1.53. Encorafenib - EMEA-001588-PIP04-23

---

Treatment of non-small cell lung cancer

Day 30 discussion

**Action:** For discussion

Oncology

### 3.1.54. Enfortomab vedotin - EMEA-002299-PIP02-23

---

Treatment of head and neck cancer

Day 30 discussion

**Action:** For discussion

Oncology

### 3.1.55. Ivonescimab - EMEA-003378-PIP01-23

---

Treatment of non-small cell lung cancer

Day 30 discussion

**Action:** For discussion

Oncology

### 3.1.56. Lerociclib - EMEA-003436-PIP01-23

---

Treatment of endometrial cancer

Day 30 discussion

**Action:** For discussion

Oncology

### 3.1.57. Modified messenger ribonucleic acid encoding individual patient-specific tumour neoantigens - EMEA-003434-PIP01-23

---

Treatment of melanoma / Treatment of lung cancer

Day 30 discussion

---

**Action:** For discussion

Oncology

### 3.1.58. [Allogeneic faecal microbiota, pooled - EMEA-003435-PIP01-23](#)

---

Treatment in allogeneic haematopoietic stem cell transplantation

Day 30 discussion

**Action:** For discussion

Oncology / Haematology-Hemostaseology

### 3.1.59. [Alpelisib - Orphan - EMEA-002016-PIP05-23](#)

---

Novartis Europharm Limited; Treatment of lymphatic malformations associated with a PIK3CA mutation

Day 30 discussion

**Action:** For discussion

Other

### 3.1.60. [Clotrimazole - EMEA-003431-PIP01-23](#)

---

Treatment of fungal otitis externa (otomycosis)

Day 30 discussion

**Action:** For discussion

Oto-rhino-laryngology

### 3.1.61. [EMEA-002990-PIP02-23](#)

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Treatment of major depressive disorder

Day 30 discussion

**Action:** For discussion

Psychiatry

### 3.1.62. [Ferric citrate coordination complex \(FCCC\) - EMEA-001213-PIP03-23](#)

---

Treatment of anaemias due to chronic kidney disorders

Day 30 discussion

**Action:** For discussion

Uro-nephrology



### 3.1.63. Ravulizumab - EMEA-001943-PIP06-23

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Prevention of death, kidney replacement therapy, or sustained reduction in kidney function in high-risk adult patients with stage 3 or 4 chronic kidney disease undergoing cardiopulmonary bypass for coronary artery bypass graft, valve replacement or repair, or combined procedure

Day 30 discussion

**Action:** For discussion

Uro-nephrology

## 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. Spesolimab - EMEA-C-002475-PIP02-19-M02

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Boehringer Ingelheim International GmbH; Treatment of generalised pustular psoriasis

Day 30 discussion

**Action:** For discussion

Dermatology

### 3.2.2. Elosulfase alfa - EMEA-C-000973-PIP01-10-M04

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BioMarin International Limited; Treatment of mucopolysaccharidosis, type IVA (Morquio A syndrome)

Day 30 discussion

**Action:** For discussion

Endocrinology-Gynaecology-Fertility-Metabolism

### 3.2.3. Saxgliptin - EMEA-C-000200-PIP01-08-M10

---

AstraZeneca AB; Treatment of type 2 diabetes

Day 30 discussion

**Action:** For discussion

Endocrinology-Gynaecology-Fertility-Metabolism

### 3.2.4. Resmetirom - EMEA-C1-003087-PIP01-21

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Madrigal Pharmaceuticals EU Limited; Treatment of non-alcoholic steatohepatitis

Day 30 discussion

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**Action:** For discussion

Gastroenterology-Hepatology

### 3.2.5. Dupilumab - EMEA-C-001501-PIP04-19-M02

---

Regeneron Ireland DAC; Treatment of eosinophilic esophagitis

Day 30 discussion

**Action:** For discussion

Gastroenterology-Hepatology

### 3.2.6. Ex vivo expanded autologous human corneal epithelial cells containing stem cells - EMEA-C-001082-PIP02-11-M03

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Holostem Therapie Avanzate S.r.l.; Treatment of limbal stem cell deficiency due to ocular burns

Day 30 discussion

**Action:** For discussion

Ophthalmology

### 3.2.7. Lanthanum carbonate hydrate - EMEA-C-000637-PIP02-10-M07

---

Takeda Pharmaceuticals International AG Ireland Branch; Treatment of hyperphosphataemia

Day 30 discussion

**Action:** For discussion

Uro-nephrology

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

### 3.3.1. Apixaban - EMEA-000183-PIP02-12-M04

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Bristol-Myers Squibb / Pfizer EEIG; Treatment of venous thromboembolism

Day 30 discussion

**Action:** For discussion

Cardiovascular Diseases

### 3.3.2. Bilayer, engineered, collagen hydrogel-based skin graft composed of autologous keratinocytes and fibroblasts - Orphan - EMEA-002699-PIP01-19-M01

---

CUTISS AG; Treatment of burns

---

Day 30 discussion

**Action:** For discussion

Dermatology

### 3.3.3. [Bimekizumab - EMEA-002189-PIP01-17-M04](#)

---

UCB Biopharma SRL; Treatment of psoriasis

Day 30 discussion

**Action:** For discussion

Dermatology

### 3.3.4. [Norucholic acid - Orphan - EMEA-002485-PIP01-18-M01](#)

---

Dr. Falk Pharma GmbH; Treatment of autoimmune sclerosing cholangitis / Treatment of primary sclerosing cholangitis

Day 30 discussion

**Action:** For discussion

Gastroenterology-Hepatology

### 3.3.5. [Risankizumab - EMEA-001776-PIP03-17-M01](#)

---

AbbVie Ltd; Treatment of Crohn's disease

Day 30 discussion

**Action:** For discussion

Gastroenterology-Hepatology

### 3.3.6. [Ustekinumab - EMEA-000311-PIP04-13-M06](#)

---

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

Day 30 discussion

**Action:** For discussion

Gastroenterology-Hepatology

### 3.3.7. [Concizumab - Orphan - EMEA-002326-PIP04-20-M01](#)

---

Novo Nordisk A/S; Treatment of congenital haemophilia A and B

Day 30 discussion

**Action:** For discussion

Haematology-Hemostaseology

### 3.3.8. Danicopan - Orphan - EMEA-002310-PIP01-17-M01

---

Alexion Europe SAS; Treatment of paroxysmal nocturnal haemoglobinuria

Day 30 discussion

**Action:** For discussion

Haematology-Hemostaseology

### 3.3.9. Etranacogene dezaparovec - Orphan - EMEA-002722-PIP01-19-M03

---

CSL Behring GmbH; Treatment of haemophilia B

Day 30 discussion

**Action:** For discussion

Haematology-Hemostaseology

### 3.3.10. Ravulizumab (ALXN1210) - Orphan - EMEA-002077-PIP01-16-M06

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Alexion Europe SAS; Treatment of paroxysmal nocturnal haemoglobinuria

Day 30 discussion

**Action:** For discussion

Haematology-Hemostaseology

### 3.3.11. Avacopan - Orphan - EMEA-002023-PIP01-16-M07

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ChemoCentryx Ireland Ltd.; Treatment of anti-neutrophil cytoplasmic auto-antibody (ANCA)-associated vasculitis

Day 30 discussion

**Action:** For discussion

Immunology-Rheumatology-Transplantation

### 3.3.12. Guselkumab - EMEA-001523-PIP03-18-M03

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Janssen-Cilag International NV; 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

### 3.3.13. Sarilumab - EMEA-001045-PIP01-10-M03

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sanofi-aventis recherche & développement; Treatment of chronic idiopathic arthritis

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(including rheumatoid arthritis, spondylarthritis, psoriatic arthritis and juvenile idiopathic arthritis)

Day 30 discussion

**Action:** For discussion

Immunology-Rheumatology-Transplantation

#### 3.3.14. Baloxavir marboxil - EMEA-002440-PIP01-18-M05

Roche Registration GmbH; Treatment of influenza / Prevention of influenza

Day 30 discussion

**Action:** For discussion

Infectious Diseases

#### 3.3.15. Fostemsavir (tromethamine) - EMEA-001687-PIP01-14-M07

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

Day 30 discussion

**Action:** For discussion

Infectious Diseases

#### 3.3.16. Tedizolid phosphate - EMEA-001379-PIP01-12-M08

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

Day 30 discussion

**Action:** For discussion

Infectious Diseases

#### 3.3.17. Tenofovir alafenamide - EMEA-001584-PIP01-13-M07

Gilead Sciences International Ltd.; Treatment of chronic hepatitis B

Day 30 discussion

**Action:** For discussion

Infectious Diseases

#### 3.3.18. Fremanezumab - EMEA-001877-PIP01-15-M04

Teva GmbH; Prevention of migraine headaches

Day 30 discussion

**Action:** For discussion

Neurology

### 3.3.19. Rozanolixizumab - Orphan - EMEA-002681-PIP01-19-M02

UCB Pharma S.A; Treatment of myasthenia gravis

Day 30 discussion

**Action:** For discussion

Neurology

### 3.3.20. Bosutinib - EMEA-000727-PIP01-09-M07

Pfizer Europe MA EEIG; Treatment of chronic myeloid leukaemia

Day 30 discussion

**Action:** For discussion

Oncology

### 3.3.21. Brexucabtagene autoleucel - Orphan - EMEA-001862-PIP01-15-M03

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

Day 30 discussion

**Action:** For discussion

Oncology

### 3.3.22. Nivolumab - EMEA-001407-PIP02-15-M06

Bristol-Myers Squibb Pharma EEIG; Treatment of malignant neoplasms of the central nervous system / Treatment of malignant neoplasms of lymphoid tissue

Day 30 discussion

**Action:** For discussion

Oncology

### 3.3.23. Mexiletine hydrochloride - Orphan - EMEA-002012-PIP01-16-M04

Lupin Europe GmbH; Treatment of myotonic disorders

Day 30 discussion

**Action:** For discussion

Other

### 3.3.24. Recombinant human ectonucleotide pyrophosphatase/phosphodiesterase 1 fused to the Fc fragment of IgG1 - Orphan - EMEA-003232-PIP01-22-M01

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

Day 30 discussion

**Action:** For discussion

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

### 3.3.25. Ravulizumab (ALXN1210) - Orphan - EMEA-001943-PIP01-16-M08

---

Alexion Europe SAS; Treatment of atypical haemolytic uremic syndrome

Day 30 discussion

**Action:** For discussion

Uro-nephrology

### 3.3.26. Davesomeran / imelasomeran / elasomeran - EMEA-002893-PIP01-20-M04

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Moderna Biotech Spain, S.L.; Prevention of coronavirus disease 2019 (COVID-19)

Day 30 discussion

**Action:** For discussion

Vaccines

### 3.3.27. Recombinant SARS-CoV-2 spike protein - EMEA-002915-PIP01-20-M02

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Sanofi Pasteur; Prevention of coronavirus disease 2019 (COVID-19)

Day 30 discussion

**Action:** For discussion

Vaccines

## 4. Nominations

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

### 4.1. List of submissions of applications with start of procedure 10 July 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.

# 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. Allogeneic adipose-derived mesenchymal stem cells - EMEA-04-2023

Magellan Stem Cells Pty Ltd; All classes of medicinal products for treatment of primary and secondary osteoarthritis / Treatment of osteoarthritis of the knee and hip

**Action:** For adoption

### 6.1.2. Nanrilkefusp alfa - EMEA-05-2023

SOTIO Biotech AG; The class of immunomodulatory cytokine medicinal products for treatment of skin malignant neoplasms / Treatment of skin squamous-cell carcinoma (alone or in combination with pembrolizumab)

**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) - Uppsala, 7-8 June 2023

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Reflections on the SRLM meeting held during the Swedish Presidency

**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

9.3.4. Upcoming Innovation Task Force (ITF) meetings

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**Action:** For information

## 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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No item

## 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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Q2/2023 Update of the Business Pipeline report for the human scientific committees

**Action:** For information

# 10. Any other business

## 10.1. DARWIN EU ® update

**Action:** For information

## **10.2. Update on the Reflection Paper on Acute Kidney Injury (AKI)**

PDCO members: Dimitar Roussinov, Sylvie Benchetrit

**Action:** For information

## **10.3. Updated scientific document and key elements form**

**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 Wednesday, 13:00 - 14:00, room 2A

## 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/)