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SCIENCE MEDICINES HEALTH

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Human Medicines Division

## Paediatric Committee (PDCO)

### Draft Agenda for the meeting on 05-08 September 2023

Chair: Brian Aylward – Vice-Chair: Sylvie Benchetrit

05 September 2023, 14:00 - 19:45, room 2C

06 September 2023, 08:30 - 19:45, room 2C

07 September 2023, 08:30 - 19:45, room 2C

08 September 2023, 08:30 - 13:00, room 2C

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#### 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 05-08 September 2023. See September 2023 PDCO minutes (to be published post October 2023 PDCO meeting).

### 1.2. Adoption of agenda

PDCO agenda for 05-08 September 2023.

### 1.3. Adoption of the minutes

PDCO minutes for 18-21 July 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. A self-complementary adeno-associated virus [AAV] serotype 8 virus particle encoding the human ornithine transcarbamylase [OTC] gene sequence - Orphan - EMEA-002830-PIP01-20

---

Ultragenyx Germany GmbH; Treatment of ornithine transcarbamylase deficiency

Day 120 opinion

**Action:** For adoption

Endocrinology-Gynaecology-Fertility-Metabolism

#### 2.1.2. A nonreplicating, recombinant adeno-associated virus (AAV) serotype 9 (AAV9) gene transfer vector that contains a modified human ATP7B coding sequence - Orphan - EMEA-003131-PIP01-21

---

Ultragenyx Germany GmbH; Treatment of Wilson disease

Day 120 opinion

**Action:** For adoption

Endocrinology-Gynaecology-Fertility-Metabolism

---

*Note: Withdrawal request received on 24 August 2023*

### 2.1.3. Fazirsiran - Orphan - EMEA-003355-PIP01-22

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

Day 120 opinion

**Action:** For adoption

Gastroenterology-Hepatology

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

Treatment of ulcerative colitis

Day 120 opinion

**Action:** For adoption

Gastroenterology-Hepatology

### 2.1.5. Tezepelumab - EMEA-001613-PIP03-21

Treatment of eosinophilic esophagitis

Day 120 opinion

**Action:** For adoption

Gastroenterology-Hepatology

### 2.1.6. EMEA-003090-PIP02-22

Treatment of hereditary angioedema

Day 120 opinion

**Action:** For adoption

Haematology-Hemostaseology

### 2.1.7. Ciraparantag - EMEA-003321-PIP01-22

Treatment of FXa inhibitor-associated haemorrhage / Prevention of FXa inhibitor-associated haemorrhage

Day 120 opinion

**Action:** For adoption

Haematology-Hemostaseology

### 2.1.8. Luspatercept - Orphan - EMEA-001521-PIP03-22

---

Bristol-Myers Squibb Pharma EEIG; Treatment of alpha-thalassaemia

Day 120 opinion

**Action:** For adoption

Haematology-Hemostaseology

### 2.1.9. Enpatoran - EMEA-003342-PIP02-22

---

Treatment of systemic lupus erythematosus

Day 120 opinion

**Action:** For adoption

Immunology-Rheumatology-Transplantation

### 2.1.10. Haematopoietic stem cells and blood progenitors umbilical cord-derived expanded with (1R,4R)-N1-(2-benzyl-7-(2-methyl-2H-tetrazol-5-yl)-9H-pyrimido[4,5-b]indol-4-yl)cyclohexane-1,4-diamine dihydrobromide dihydrate - Orphan - EMEA-003025-PIP03-23

---

ExCellThera; Treatment in haematopoietic stem cell transplantation in patients with acute myeloid leukaemia

Day 120 opinion

**Action:** For adoption

Immunology-Rheumatology-Transplantation / Oncology / Haematology-Hemostaseology

### 2.1.11. Ensitrelvir - EMEA-003192-PIP01-22

---

Treatment of coronavirus disease 2019 (COVID-19)

Day 120 opinion

**Action:** For adoption

Infectious Diseases

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

---

Treatment of chronic hepatitis B

Day 120 opinion

**Action:** For adoption

Infectious Diseases

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

---

Treatment of chronic hepatitis B

Day 120 opinion

**Action:** For adoption

Infectious Diseases

#### 2.1.14. Rozanolixizumab - EMEA-002681-PIP03-21

---

Treatment of myelin oligodendrocyte glycoprotein antibody-associated disease

Day 120 opinion

**Action:** For adoption

Neurology

#### 2.1.15. Nemvaleukin alfa - EMEA-003357-PIP01-22

---

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

Day 120 opinion

**Action:** For adoption

Oncology

#### 2.1.16. Pembrolizumab / vibostolimab - EMEA-003063-PIP03-22

---

Treatment of melanoma

Day 120 opinion

**Action:** For adoption

Oncology

#### 2.1.17. Uproleselan - Orphan - EMEA-003307-PIP01-22

---

GlycoMimetics, Inc.; Treatment of acute myeloid leukaemia

Day 120 opinion

**Action:** For adoption

Oncology

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

---

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

---

Day 120 opinion

**Action:** For adoption

Other

---

#### 2.1.19. EMEA-003347-PIP01-22

---

Treatment of glomerulonephritis and nephrotic syndrome

Day 120 opinion

**Action:** For adoption

Uro-nephrology

---

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

---

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

Day 120 opinion

**Action:** For adoption

Uro-nephrology

#### 2.1.21. 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 120 opinion

**Action:** For adoption

Vaccines

#### 2.1.22. *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

---

Prevention of meningococcal disease

---

Day 120 opinion

**Action:** For adoption

Vaccines

---

#### 2.1.23. [Amlodipine / rosuvastatin - EMEA-003446-PIP01-23](#)

---

Treatment of angina and dyslipidaemia / Treatment of essential hypertension in patients who are estimated to have a high risk for a first cardiovascular event / Treatment of concomitant hypertension and dyslipidemia

Day 60 opinion

**Action:** For adoption

Cardiovascular Diseases

---

#### 2.1.24. [Modified, recombinant version of the human myeloid-derived growth factor - EMEA-003449-PIP01-23](#)

---

Treatment of myocardial infarction

Day 60 opinion

**Action:** For adoption

Cardiovascular Diseases

---

#### 2.1.25. [EMEA-003456-PIP01-23](#)

---

Treatment of myelofibrosis

Day 60 opinion

**Action:** For adoption

Haematology-Hemostaseology

---

#### 2.1.26. [EMEA-003455-PIP01-23](#)

---

Treatment of lung cancer

Day 60 opinion

**Action:** For adoption

Oncology

---

#### 2.1.27. [Belrestotug - EMEA-003452-PIP01-23](#)

---

Treatment of lung cancer

Day 60 opinion

**Action:** For adoption

Oncology

#### 2.1.28. [Bezuclastinib - EMEA-003445-PIP01-23](#)

---

Treatment of mastocytosis

Day 60 opinion

**Action:** For adoption

Oncology

#### 2.1.29. [Humanised IgG1 monoclonal antibody against TROP2, conjugated to a topoisomerase I inhibitor belotecan-derivative - EMEA-003461-PIP01-23](#)

---

Treatment of lung cancer / Treatment of ovarian cancer / Treatment of cervical cancer /  
Treatment of gastric cancer / Treatment of breast cancer / Treatment of endometrial cancer

Day 60 opinion

**Action:** For adoption

Oncology

#### 2.1.30. [Tiragolumab - EMEA-002721-PIP04-23](#)

---

Treatment of hepatocellular carcinoma

Day 60 opinion

**Action:** For adoption

Oncology

#### 2.1.31. [Valemetostat tosilate - Orphan - EMEA-003256-PIP02-23](#)

---

Daiichi Sankyo Europe GmbH; Treatment of mature T-cell neoplasms

Day 60 opinion

**Action:** For adoption

Oncology

#### 2.1.32. [Zanidatamab - Orphan - EMEA-003450-PIP01-23](#)

---

Jazz Pharmaceuticals Ireland Limited; Treatment of biliary tract cancer (BTC)

Day 60 opinion

**Action:** For adoption

Oncology

2.1.33. Pertussis filamentous haemagglutinin (FHA) / Genetically detoxified pertussis toxin (PTgen) - EMEA-003442-PIP01-23

---

Prevention of pertussis disease

Day 60 opinion

**Action:** For adoption

Vaccines

## 2.2. Opinions on Compliance Check

2.2.1. Abacavir (ABC) / lamivudine (3TC) / dolutegravir (DTG) - EMEA-C-001219-PIP01-11-M06

---

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

Day 60 opinion

**Action:** For adoption

Infectious Diseases

2.2.2. Leriglitazone - EMEA-C-002106-PIP01-16-M02

---

Minoryx Therapeutics S.L.; Treatment of adrenoleukodystrophy

Day 60 opinion

**Action:** For adoption

Neurology

2.2.3. Molgramostim - EMEA-C1-002282-PIP01-17-M01

---

Savara ApS; Treatment of pulmonary alveolar proteinosis

Day 60 letter

**Action:** For adoption

Pneumology – Allergology

2.2.4. Bulevirtide - EMEA-C-002399-PIP01-18-M01

---

Gilead Sciences Ireland UC; Treatment of chronic hepatitis D infection

Day 30 opinion

**Action:** For adoption

Infectious Diseases



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

### 2.3.1. Dupilumab - EMEA-001501-PIP02-13-M08

---

Sanofi Winthrop Industrie; Treatment of asthma

Day 60 opinion

**Action:** For adoption

Dermatology

### 2.3.2. Fitusiran - Orphan - EMEA-001855-PIP01-15-M05

---

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

Day 60 opinion

**Action:** For adoption

Haematology-Hemostaseology

### 2.3.3. Vonicog alfa - EMEA-001164-PIP01-11-M07

---

Baxalta Innovations GmbH; Treatment of Von Willebrand disease

Day 60 opinion

**Action:** For adoption

Haematology-Hemostaseology

### 2.3.4. BNT162b2 / tozinameran / famtozinameran / riltozinameran - EMEA-002861-PIP02-20-M06

---

BioNTech Manufacturing GmbH; Prevention of coronavirus disease 2019 (COVID-19)

Day 60 opinion

**Action:** For adoption

Infectious Diseases

### 2.3.5. Eravacycline - EMEA-001555-PIP01-13-M05

---

PAION Deutschland GmbH; Treatment of complicated intra-abdominal infection

Day 60 opinion

**Action:** For adoption

Infectious Diseases

### 2.3.6. Remdesivir - EMEA-002826-PIP01-20-M04

---

Gilead Sciences International Ltd.; Treatment of coronavirus disease 2019 (COVID-19)

Day 60 opinion

**Action:** For adoption

Infectious Diseases

### 2.3.7. Eptinezumab - EMEA-002243-PIP01-17-M04

---

H. Lundbeck A/S; Prevention of migraine headaches

Day 60 opinion

**Action:** For adoption

Neurology

### 2.3.8. Nipocalimab (anti-neonatal Fc receptor human monoclonal antibody) - EMEA-002559-PIP02-19-M01

---

Janssen-Cilag International NV; Treatment of myasthenia gravis

Day 60 opinion

**Action:** For adoption

Neurology

### 2.3.9. Onasemnogene abeparvovec - Orphan - EMEA-002168-PIP01-17-M05

---

Novartis Europharm Limited; Treatment of spinal muscular atrophy

Day 60 opinion

**Action:** For adoption

Neurology

### 2.3.10. Peginterferon beta-1a - EMEA-001129-PIP01-11-M06

---

Biogen Idec Ltd; Treatment of multiple sclerosis

Day 60 opinion

**Action:** For adoption

Neurology

### 2.3.11. Carfilzomib - Orphan - EMEA-001806-PIP04-19-M02

---

Amgen Europe B.V; Treatment of acute lymphoblastic leukaemia

Day 60 opinion

---

**Action:** For adoption

Oncology

#### 2.3.12. Cemiplimab - EMEA-002007-PIP02-17-M03

Regeneron Ireland DAC; Treatment of all conditions included in the category of malignant neoplasms (except haematopoietic and lymphoid tissue)

Day 60 opinion

**Action:** For adoption

Oncology

#### 2.3.13. Tovorafenib - Orphan - EMEA-002763-PIP01-20-M01

Day One Biopharmaceuticals, Inc; Treatment of paediatric low-grade glioma

Day 60 opinion

**Action:** For adoption

Oncology

#### 2.3.14. Iptacopan - Orphan - EMEA-002705-PIP02-19-M01

Novartis Europharm Limited; Treatment of IgA nephropathy

Day 60 opinion

**Action:** For adoption

Other

#### 2.3.15. Ivacaftor / tezacaftor / elexacaftor - Orphan - EMEA-002324-PIP01-17-M05

Vertex Pharmaceuticals (Ireland) Limited; Treatment of cystic fibrosis

Day 60 opinion

**Action:** For adoption

Other

#### 2.3.16. Palonosetron / fosnetupitant - EMEA-001198-PIP03-17-M05

Helsinn Birex Pharmaceuticals Limited; Prevention of chemotherapy-induced nausea and vomiting

Day 60 opinion

**Action:** For adoption

Other

### 2.3.17. Selexipag - EMEA-000997-PIP01-10-M07

---

Janssen-Cilag International NV; Treatment of pulmonary arterial hypertension

Day 60 opinion

**Action:** For adoption

Other

### 2.3.18. Evenamide - EMEA-002519-PIP03-21-M01

---

Newron Pharmaceuticals S.p.A.; Treatment of schizophrenia

Day 60 opinion

**Action:** For adoption

Psychiatry

### 2.3.19. NVX-CoV2373 - EMEA-002941-PIP01-20-M04

---

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

Day 60 opinion

**Action:** For adoption

Vaccines

### 2.3.20. Recombinant COVID-19 subunit nanoparticle - EMEA-003115-PIP01-21-M02

---

SK Chemicals GmbH; Prevention of coronavirus disease 2019 (COVID-19)

Day 60 opinion

**Action:** For adoption

Vaccines

### 2.3.21. Autologous tumour-infiltrating lymphocytes (LN-144/LN-145) - EMEA-002776-PIP01-20-M02

---

Iovance Biotherapeutics, Inc.; Treatment of all conditions included in the category of malignant neoplasms (except haematopoietic and lymphoid tissue neoplasms)

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. Beremagene geperpavec - EMEA-C1-002472-PIP03-22-M01

---

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

Day 30 letter

**Action:** For information

Dermatology

### 2.7.2. Gepotidacin - EMEA-C1-002443-PIP01-18-M02

---

GlaxoSmithKline Trading Services Limited; Treatment of uncomplicated urinary tract infections

Day 30 letter

**Action:** For information

Infectious Diseases

## 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. Amlitelimab - EMEA-003233-PIP01-22

---

Treatment of atopic dermatitis

Day 90 discussion

**Action:** For discussion

Dermatology

---

### 3.1.2. Upadacitinib - EMEA-001741-PIP08-22

---

Treatment of hidradenitis suppurativa

Day 90 discussion

**Action:** For discussion

Dermatology

### 3.1.3. Upadacitinib - EMEA-001741-PIP09-23

---

Treatment of systemic lupus erythematosus

Day 90 discussion

**Action:** For discussion

Dermatology

### 3.1.4. *Escherichia coli*, expressing high affinity phenylalanine transporter, modified phenylalanine ammonia lyase and L-amino acid deaminase - EMEA-003381-PIP01-22

---

Treatment of hyperphenylalaninemia

Day 90 discussion

**Action:** For discussion

Endocrinology-Gynaecology-Fertility-Metabolism

### 3.1.5. Recombinant human monoclonal antibody to insulin receptor - Orphan - EMEA-002813-PIP01-23

---

Rezolute (Bio) Ireland Limited; Treatment of congenital hyperinsulinism

Day 90 discussion

**Action:** For discussion

Endocrinology-Gynaecology-Fertility-Metabolism

### 3.1.6. Venglustat - Orphan - EMEA-001716-PIP07-22

---

Sanofi B.V.; Treatment of Gaucher disease type 3 / Treatment of Gaucher disease

Day 90 discussion

**Action:** For discussion

Endocrinology-Gynaecology-Fertility-Metabolism

### 3.1.7. Cemdisiran - Orphan - EMEA-003237-PIP02-22

---

Regeneron Ireland DAC; Treatment of paroxysmal nocturnal haemoglobinuria

Day 90 discussion

**Action:** For discussion

Haematology-Hemostaseology

### 3.1.8. Pozelimab - EMEA-003238-PIP02-22

---

Treatment of paroxysmal nocturnal haemoglobinuria

Day 90 discussion

**Action:** For discussion

Haematology-Hemostaseology

### 3.1.9. Tetrahydrouridine / decitabine - Orphan - EMEA-003404-PIP01-23

---

Novo Nordisk A/S; Treatment of sickle cell disease (SCD)

Day 90 discussion

**Action:** For discussion

Haematology-Hemostaseology

### 3.1.10. Bidridistrogene xeboparvovec - Orphan - EMEA-003400-PIP01-23

---

Sarepta Therapeutics Ireland; Treatment of limb-girdle muscular dystrophy

Day 90 discussion

**Action:** For discussion

Neurology

### 3.1.11. Inebilizumab - EMEA-001911-PIP02-22

---

Treatment of generalised myasthenia gravis

Day 90 discussion

**Action:** For discussion

Neurology

### 3.1.12. Cobolimab - EMEA-003273-PIP02-22

---

Treatment of all conditions included in the category of malignant neoplasms including lymphoma (except lung cancers and hematopoietic malignancies)

Day 90 discussion

**Action:** For discussion

Oncology

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

---

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

Day 90 discussion

**Action:** For discussion

Other

### 3.1.14. Oral inhibitor of PCSK9 - EMEA-003453-PIP01-23

---

Treatment of hypercholesterolemia

Day 60 discussion

**Action:** For discussion

Cardiovascular Diseases

### 3.1.15. Upadacitinib - EMEA-001741-PIP10-23

---

Treatment of alopecia areata

Day 60 discussion

**Action:** For discussion

Dermatology

### 3.1.16. GIPR antagonist/GLP-1R agonist - EMEA-003439-PIP02-23

---

Treatment of obesity

Day 60 discussion

**Action:** For discussion

Endocrinology-Gynaecology-Fertility-Metabolism

### 3.1.17. Plecanatide - EMEA-003441-PIP01-23

---

Treatment of irritable bowel syndrome with constipation / Treatment of chronic idiopathic constipation

Day 60 discussion

**Action:** For discussion



Gastroenterology-Hepatology

### 3.1.18. Hydroxycarbamide - EMEA-003388-PIP01-23

---

Prevention of sickle cell disease crises

Day 60 discussion

**Action:** For discussion

Haematology-Hemostaseology

### 3.1.19. Ianalumab - EMEA-002338-PIP04-23

---

Treatment of autoimmune haemolytic anaemia

Day 60 discussion

**Action:** For discussion

Haematology-Hemostaseology

### 3.1.20. Reparixin - Orphan - EMEA-001693-PIP06-23

---

Dompé farmaceutici S.p.A.; Treatment of infectious pneumonia acquired in the community, excluding coronavirus disease 2019 (COVID-19)

Day 60 discussion

**Action:** For discussion

Infectious Diseases

### 3.1.21. Ganaxolone - EMEA-002341-PIP03-23

---

Treatment of refractory status epilepticus

Day 60 discussion

**Action:** For discussion

Neurology

### 3.1.22. Recombinant adeno-associated virus Olig001 containing human aspartoacylase cDNA - Orphan - EMEA-003459-PIP01-23

---

Myrtelle,Inc.; Treatment of Canavan disease

Day 60 discussion

**Action:** For discussion

Neurology

### 3.1.23. Autologous patient-derived CD4+ and CD8+ T cells expressing a chimeric antigen receptor specific for claudin 6 - EMEA-003377-PIP01-23

---

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

Day 60 discussion

**Action:** For discussion

Oncology

### 3.1.24. Disitamab vedotin - EMEA-003443-PIP02-23

---

Treatment of HER2 expressing tumours / Treatment of solid tumours

Day 60 discussion

**Action:** For discussion

Oncology

### 3.1.25. Liposome-formulated messenger ribonucleic-acid vaccine encoding the chimeric antigen-receptor target antigen claudin 6 - EMEA-003464-PIP01-23

---

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

Day 60 discussion

**Action:** For discussion

Oncology

### 3.1.26. Obecabtagene autoleucel - Orphan - EMEA-003171-PIP02-23

---

Autolus GmbH; Treatment of non-Hodgkin lymphoma

Day 60 discussion

**Action:** For discussion

Oncology

### 3.1.27. Unesbulin - Orphan - EMEA-003297-PIP02-23

---

PTC Therapeutics International; Treatment of soft tissue sarcoma

Day 60 discussion

**Action:** For discussion

Oncology

### 3.1.28. Allogeneic faecal microbiota, pooled - Orphan - EMEA-003435-PIP02-23

---

MaaT Pharma; Treatment of graft-versus-host disease

Day 60 discussion

**Action:** For discussion

Oncology / Haematology-Hemostaseology

### 3.1.29. Clobetasol - EMEA-003458-PIP01-23

---

Treatment of inflammation and pain associated with ocular surgery

Day 60 discussion

**Action:** For discussion

Ophthalmology

### 3.1.30. Laruparetigene zovaparvovec - Orphan - EMEA-003457-PIP01-23

---

FGK Representative Service GMBH; Treatment of X-linked retinitis pigmentosa

Day 60 discussion

**Action:** For discussion

Ophthalmology

### 3.1.31. Garetosmab - Orphan - EMEA-002736-PIP02-23

---

Regeneron Ireland DAC; Treatment of fibrodysplasia ossificans progressiva

Day 60 discussion

**Action:** For discussion

Other

### 3.1.32. Losmapimod - Orphan - EMEA-003448-PIP01-23

---

Fulcrum Therapeutics, Inc.; Treatment of facioscapulohumeral muscular dystrophy

Day 60 discussion

**Action:** For discussion

Other

### 3.1.33. Mannose-1-phosphate - Orphan - EMEA-003460-PIP01-23

---

Glycomine Inc.; Treatment of phosphomannomutase 2-congenital disorder of glycosylation

Day 60 discussion

**Action:** For discussion

Other

### 3.1.34. [Sisunatovir - EMEA-002529-PIP02-23](#)

---

Treatment of respiratory tract disease caused by respiratory syncytial virus (RSV) infection

Day 60 discussion

**Action:** For discussion

Other

### 3.1.35. [Imlifidase - EMEA-002183-PIP02-23](#)

---

Treatment of patients with Duchenne muscular dystrophy (DMD) and pre-existing antibodies to the AAV vector to enable gene therapy

Day 60 discussion

**Action:** For discussion

Other / Uro-nephrology

### 3.1.36. [Mometasone - EMEA-003454-PIP01-23](#)

---

Treatment of chronic rhinosinusitis (CRS)

Day 60 discussion

**Action:** For discussion

Oto-rhino-laryngology

### 3.1.37. [EMEA-003451-PIP01-23](#)

---

Treatment of bronchiectasis

Day 60 discussion

**Action:** For discussion

Pneumology - Allergology

### 3.1.38. [Borrelia outer surface protein A \(OspA\) serotypes \(ST1-6\) lipidated, fusion protein vaccine - EMEA-003130-PIP02-23](#)

---

Prevention of Lyme disease

Day 60 discussion

**Action:** For discussion

Vaccines

### 3.1.39. Candesartan / atorvastatin / amlodipine - EMEA-003466-PIP01-23

---

Prevention of cardiovascular events / Treatment of dyslipidaemia / Treatment of hypertension

Day 30 discussion

**Action:** For discussion

Cardiovascular Diseases

### 3.1.40. Ezetimibe / rosuvastatin - EMEA-003472-PIP01-23

---

Treatment of hypercholesterolaemia / Prevention of cardiovascular events

Day 30 discussion

**Action:** For discussion

Cardiovascular Diseases

### 3.1.41. Fusion protein consisting of relaxin and Fc domain of IgG1 - EMEA-003476-PIP01-23

---

Treatment of pulmonary hypertension due to left heart disease (PH-LHD, WHO group 2 PH)

Day 30 discussion

**Action:** For discussion

Cardiovascular Diseases

### 3.1.42. Glyceryl - EMEA-003479-PIP01-23

---

Treatment of coronary artery disorders / Treatment of heart failure signs and symptoms / Prevention of cardiac and vascular procedural complications

Day 30 discussion

**Action:** For discussion

Cardiovascular Diseases

### 3.1.43. EMEA-003480-PIP01-23

---

Treatment of psoriasis

Day 30 discussion

**Action:** For discussion

Dermatology

#### 3.1.44. Lutikizumab - EMEA-003481-PIP01-23

---

Treatment of hidradenitis suppurativa

Day 30 discussion

**Action:** For discussion

Dermatology

#### 3.1.45. EMEA-003478-PIP01-23

---

Treatment of psoriasis

Day 30 discussion

**Action:** For discussion

Dermatology

#### 3.1.46. Bitopertin - Orphan - EMEA-000439-PIP03-23

---

Disc Medicine B.V.; Treatment of erythropoietic protoporphyria, including X-linked-  
protoporphyria

Day 30 discussion

**Action:** For discussion

Dermatology / Haematology-Hemostaseology

#### 3.1.47. Etavopivat - Orphan - EMEA-002924-PIP02-23

---

Novo Nordisk A/S; Treatment of sickle cell disease

Day 30 discussion

**Action:** For discussion

Haematology-Hemostaseology

#### 3.1.48. Human alpha-1 proteinase inhibitor, modified - EMEA-003463-PIP01-23

---

Treatment of haemophilia B

Day 30 discussion

**Action:** For discussion

Haematology-Hemostaseology

#### 3.1.49. Ianalumab - EMEA-002338-PIP05-23

---

Treatment of immune thrombocytopenia (ITP)

Day 30 discussion

**Action:** For discussion

Haematology-Hemostaseology

---

### 3.1.50. EMEA-003002-PIP04-23

---

Treatment of systemic sclerosis (SSc)

Day 30 discussion

**Action:** For discussion

Immunology-Rheumatology-Transplantation

---

### 3.1.51. Iodine (<sup>131</sup>I) apamistamab - Orphan - EMEA-003395-PIP02-23

---

Immedica Pharma AB; Treatment in allogenic stem cell transplantation

Day 30 discussion

**Action:** For discussion

Immunology-Rheumatology-Transplantation / Oncology

---

### 3.1.52. Radiprodil - EMEA-003462-PIP01-23

---

Treatment of GRIN-related disorders

Day 30 discussion

**Action:** For discussion

Neurology

---

### 3.1.53. EMEA-003477-PIP01-23

---

Treatment of appetite and nutrition disorders

Day 30 discussion

**Action:** For discussion

Nutrition

---

### 3.1.54. (3S,3'S,3a'S,10a'S)-6-chloro-3'-(3-chloro-2-fluorophenyl)-1'-(cyclopropylmethyl)-6'-methyl-2-oxo-1,2,3',3a',10',10a'-hexahydro-1'H-spiro[indole-3,2'-pyrrolo[2',3':4,5]pyrrolo[1,2-b]indazole]-7'-carboxylic acid - Orphan - EMEA-003260-PIP03-23

---

Boehringer Ingelheim International GmbH; Treatment of soft tissue sarcoma

Day 30 discussion

**Action:** For discussion

---

Oncology

### 3.1.55. Actinium n.c.a - EMEA-003482-PIP01-23

---

Radiolabelling agent

Day 30 discussion

**Action:** For discussion

Oncology

### 3.1.56. EMEA-003470-PIP01-23

---

Treatment of breast malignant neoplasms

Day 30 discussion

**Action:** For discussion

Oncology

### 3.1.57. Gotistobart - EMEA-003467-PIP01-23

---

Treatment of non-small cell lung cancer

Day 30 discussion

**Action:** For discussion

Oncology

### 3.1.58. Humanised IgG1 monoclonal antibody against integrin beta-6 conjugated to monomethyl auristatin E via a valine-citrulline linker - EMEA-003471-PIP01-23

---

Treatment of solid tumours

Day 30 discussion

**Action:** For discussion

Oncology

### 3.1.59. Ilginatnib - EMEA-003468-PIP01-23

---

Treatment of myelofibrosis

Day 30 discussion

**Action:** For discussion

Oncology



### 3.1.60. Relatlimab / nivolumab - EMEA-002727-PIP02-23

---

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

Day 30 discussion

**Action:** For discussion

Oncology

### 3.1.61. Relatlimab / nivolumab - EMEA-002727-PIP03-23

---

Treatment of melanoma

Day 30 discussion

**Action:** For discussion

Oncology

### 3.1.62. EMEA-003469-PIP01-23

---

Treatment of non-small cell lung cancer / Treatment of non-small cell lung carcinoma

Day 30 discussion

**Action:** For discussion

Other

### 3.1.63. Messenger RNA encoding Cas9, single guide RNA targeting the human KLKB1 gene - EMEA-003465-PIP01-23

---

Treatment of hereditary angioedema (HAE)

Day 30 discussion

**Action:** For discussion

Other

### 3.1.64. Ulviprubart - EMEA-003474-PIP01-23

---

Treatment of inclusion body myositis

Day 30 discussion

**Action:** For discussion

Other / Immunology-Rheumatology-Transplantation

### 3.1.65. Imlifidase - Orphan - EMEA-002183-PIP03-23

---

Hansa Biopharma AB; Treatment of anti-glomerular basement membrane (anti-GBM)

---

disease

Day 30 discussion

**Action:** For discussion

Other / Uro-nephrology

### 3.1.66. [Naproxen / paracetamol - EMEA-003475-PIP01-23](#)

---

Treatment of rheumatoid arthritis / Treatment of febrile disorders / Treatment of acute pain

Day 30 discussion

**Action:** For discussion

Pain

### 3.1.67. [Humanised IgG1 monoclonal antibody against pituitary adenylate cyclase-activating polypeptide - EMEA-003483-PIP01-23](#)

---

Prevention of migraine

Day 30 discussion

**Action:** For discussion

Pain / Neurology

### 3.1.68. [Atacicept - EMEA-002004-PIP04-23](#)

---

Treatment immunoglobulin A nephropathy

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. [Macitentan - EMEA-C3-001032-PIP01-10-M05](#)

---

Janssen-Cilag International NV; Treatment of pulmonary arterial hypertension

Day 30 discussion

**Action:** For discussion

Cardiovascular Diseases

### 3.2.2. Remibrutinib - EMEA-C1-002582-PIP01-19-M02

---

Novartis Europharm Limited; Treatment of chronic spontaneous urticaria

Day 30 discussion

**Action:** For discussion

Dermatology

### 3.2.3. Sarilumab - EMEA-C1-001045-PIP01-10-M03

---

sanofi-aventis recherche & développement; Treatment of chronic idiopathic arthritis (including rheumatoid arthritis, spondylarthritis, psoriatic arthritis and juvenile idiopathic arthritis)

Day 30 discussion

**Action:** For discussion

Immunology-Rheumatology-Transplantation

### 3.2.4. Nirsevimab - EMEA-C-001784-PIP01-15-M04

---

AstraZeneca AB; Prevention of lower respiratory tract infection caused by respiratory syncytial virus

Day 30 discussion

**Action:** For discussion

Infectious Diseases

### 3.2.5. Ataluren - EMEA-C-000115-PIP01-07-M13

---

PTC Therapeutics International, Limited; Treatment of dystrophinopathy

Day 30 discussion

**Action:** For discussion

Neurology

### 3.2.6. Lenvatinib - EMEA-C-001119-PIP03-19-M03

---

Eisai GmbH; Treatment of all conditions included in the category of malignant neoplasms except haematopoietic and lymphoid tissue neoplasms, papillary thyroid cancer, follicular thyroid cancer and osteosarcoma

Day 30 discussion

**Action:** For discussion

Oncology

### 3.2.7. Repotrectinib - EMEA-C1-002635-PIP02-21-M01

---

Bristol-Myers Squibb Pharma EEIG; Treatment of all conditions included in the category of malignant neoplasms (except haematopoietic neoplasms)

Day 30 discussion

**Action:** For discussion

Oncology

### 3.2.8. Cysteamine hydrochloride - EMEA-C-000322-PIP01-08-M06

---

Recordati Rare Diseases; Treatment of corneal cystine crystal deposits in cystinosis

Day 30 discussion

**Action:** For discussion

Ophthalmology

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

### 3.3.1. Angiotensin II acetate - EMEA-001912-PIP02-16-M03

---

PAION Deutschland GmbH; Treatment of hypotension associated with distributive or vasodilatory shock

Day 30 discussion

**Action:** For discussion

### 3.3.2. Macitentan - Orphan - EMEA-001032-PIP01-10-M07

---

Janssen-Cilag International NV; Treatment of pulmonary arterial hypertension / Treatment of systemic sclerosis / Treatment of idiopathic pulmonary fibrosis

Day 30 discussion

**Action:** For discussion

Cardiovascular Diseases

### 3.3.3. Treprostinil sodium - EMEA-003182-PIP01-22-M01

---

AOP Orphan Pharmaceuticals GmbH; Treatment of pulmonary arterial hypertension (PAH) group 1 / Treatment of pulmonary arterial hypertension

Day 30 discussion

**Action:** For discussion

Cardiovascular Diseases

#### 3.3.4. Abrocitinib - EMEA-002312-PIP01-17-M02

---

Pfizer Europe MA EEIG; Treatment of moderate to severe atopic dermatitis

Day 30 discussion

**Action:** For discussion

Dermatology

#### 3.3.5. Remibrutinib - EMEA-002582-PIP01-19-M03

---

Novartis Europharm Limited; Treatment of chronic spontaneous urticaria

Day 30 discussion

**Action:** For discussion

Dermatology

#### 3.3.6. Liraglutide - EMEA-000128-PIP02-09-M05

---

Novo Nordisk A/S; Treatment of obesity

Day 30 discussion

**Action:** For discussion

Endocrinology-Gynaecology-Fertility-Metabolism

#### 3.3.7. Pegzilarginase - Orphan - EMEA-001925-PIP02-19-M01

---

Aeglea BioTherapeutics Inc.; Treatment of hyperargininaemia

Day 30 discussion

**Action:** For discussion

Endocrinology-Gynaecology-Fertility-Metabolism

#### 3.3.8. Maralixibat chloride - Orphan - EMEA-001475-PIP03-17-M04

---

Mirum Pharmaceuticals; Treatment of progressive familial intrahepatic cholestasis

Day 30 discussion

**Action:** For discussion

Gastroenterology-Hepatology

#### 3.3.9. Denecimig - EMEA-002762-PIP02-20-M02

---

Novo Nordisk A/S; Treatment of haemophilia A

Day 30 discussion

**Action:** For discussion

Haematology-Hemostaseology

### 3.3.10. Human fibrinogen concentrate - EMEA-001931-PIP01-16-M03

Biotest AG; Treatment of congenital fibrinogen deficiency

Day 30 discussion

**Action:** For discussion

Haematology-Hemostaseology

### 3.3.11. Sebetralstat - Orphan - EMEA-002723-PIP01-19-M02

KalVista Pharmaceuticals Ltd; Treatment of hereditary angioedema

Day 30 discussion

**Action:** For discussion

Haematology-Hemostaseology

### 3.3.12. Risankizumab – EMEA-001776-PIP02-17-M02

AbbVie Ltd; Treatment of chronic idiopathic arthritis

Day 30 discussion

**Action:** For discussion

Immunology-Rheumatology-Transplantation

### 3.3.13. Upadacitinib - EMEA-001741-PIP03-16-M04

AbbVie Ltd; Treatment of Crohn's disease

Day 30 discussion

**Action:** For discussion

Immunology-Rheumatology-Transplantation / Gastroenterology-Hepatology

### 3.3.14. Bemnifosbuvir - EMEA-002963-PIP01-21-M01

Atea Pharmaceuticals, Inc; Treatment of coronavirus disease 2019 (COVID-19)

Day 30 discussion

**Action:** For discussion

Infectious Diseases

### 3.3.15. Cilgavimab (AZD1061) - EMEA-002925-PIP01-20-M02

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

Day 30 discussion

**Action:** For discussion

Infectious Diseases

### 3.3.16. Dalbavancin hydrochloride - EMEA-000016-PIP01-07-M09

AbbVie Ltd; Treatment of acute bacterial skin and skin structure infections (ABSSSI)

Day 30 discussion

**Action:** For discussion

Infectious Diseases

### 3.3.17. Letemovir - Orphan - EMEA-001631-PIP01-14-M05

Merck Sharp & Dohme (Europe), Inc.; Prevention of cytomegalovirus infection

Day 30 discussion

**Action:** For discussion

Infectious Diseases

### 3.3.18. Relebactam monohydrate / cilastatin sodium / imipenem monohydrate - EMEA-001809-PIP01-15-M04

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

Day 30 discussion

**Action:** For discussion

Infectious Diseases

### 3.3.19. Ritonavir / nirmatrelvir - EMEA-003081-PIP01-21-M03

Pfizer Europe MA EEIG; Treatment of coronavirus disease 2019 (COVID-19) / Prevention of coronavirus disease 2019 (COVID-19)

Day 30 discussion

**Action:** For discussion

Infectious Diseases

### 3.3.20. Tixagevimab (AZD8895) - EMEA-002900-PIP01-20-M02

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

Day 30 discussion

**Action:** For discussion

Infectious Diseases

### 3.3.21. Zanamivir - EMEA-001318-PIP01-12-M05

---

GlaxoSmithKline Trading Services Limited; Treatment of influenza / Prevention of influenza

Day 30 discussion

**Action:** For discussion

Infectious Diseases

### 3.3.22. Alprazolam - EMEA-003043-PIP01-21-M01

---

UCB Pharma SA.; Treatment of epileptic seizures

Day 30 discussion

**Action:** For discussion

Neurology

### 3.3.23. Brivaracetam - EMEA-000332-PIP02-17-M05

---

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

Day 30 discussion

**Action:** For discussion

Neurology

### 3.3.24. Ravulizumab - EMEA-001943-PIP04-20-M01

---

Alexion Europe SAS; Treatment of neuromyelitis optica spectrum disorders

Day 30 discussion

**Action:** For discussion

Neurology

### 3.3.25. Satralizumab - Orphan - EMEA-001625-PIP02-21-M02

---

Roche Registration GmbH; Treatment of generalised myasthenia gravis

Day 30 discussion

**Action:** For discussion

Neurology

### 3.3.26. Blinatumomab - Orphan - EMEA-000574-PIP02-12-M04

---

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

Day 30 discussion

**Action:** For discussion



Oncology

### 3.3.27. Lisocabtagene maraleucel - EMEA-001995-PIP01-16-M04

---

Bristol-Myers Squibb Pharma EEIG; Treatment of B-lymphoblastic leukaemia/lymphoma /  
Treatment of mature B-cell neoplasms

Day 30 discussion

**Action:** For discussion

Oncology

### 3.3.28. Lutetium (<sup>177</sup>Lu) oxodotreotide - Orphan - EMEA-002950-PIP01-20-M01

---

Advanced Accelerator Applications; Treatment of gastroenteropancreatic neuroendocrine  
tumours (GEP-NETs)

Day 30 discussion

**Action:** For discussion

Oncology

### 3.3.29. Tirzepatide - EMEA-002360-PIP02-22-M02

---

Eli Lilly and Company; Treatment of obesity

Day 30 discussion

**Action:** For discussion

Other

### 3.3.30. Tapentadol - EMEA-000325-PIP01-08-M11

---

Grünenthal GmbH; Treatment of chronic pain

Day 30 discussion

**Action:** For discussion

Pain

### 3.3.31. Mometasone (furoate) / glycopyrronium bromide / indacaterol - EMEA-001812-PIP01-15-M02

---

Novartis Europharm Limited; Treatment of asthma

Day 30 discussion

**Action:** For discussion

Pneumology - Allergology

### 3.3.32. Seltorexant - EMEA-002746-PIP01-20-M02

---

Janssen-Cilag International NV; Treatment of major depressive disorder

Day 30 discussion

**Action:** For discussion

Psychiatry

## 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 11 September 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

No item

## 7. Discussion on the inclusion of an indication within a condition in an agreed PIP/waiver

### 7.1. Discussion on the possibility to include an indication within a condition in an agreed PIP/waiver

#### 7.1.1. Setmelanotide - Orphan - EMEA-002209-PIP01-17-M03

---

Rhythm Pharmaceuticals, Inc; Treatment of appetite and general nutrition disorders

Proposed indication: Treatment of hypothalamic obesity

**Action:** For adoption

#### 7.1.2. Finerenone - EMEA-001623-PIP01-14-M06

---

Bayer AG; Treatment of chronic kidney disease

Proposed indication: Treatment of chronic kidney disease in adults with type 1 diabetes

**Action:** For adoption

## 8. Annual reports on deferrals

Note: The annual reports on deferrals to be noted by the members of the PDCO are flagged in the Annex B.

## 9. Organisational, regulatory and methodological matters

### 9.1. Mandate and organisation of the PDCO

#### 9.1.1. PDCO membership

---

**Action:** For information

#### 9.1.2. Vote by Proxy

---

**Action:** For information

#### 9.1.3. Strategic Review and Learning Meeting (SRLM) - Madrid, Spain 17-18 October 2023

---

**Action:** For information

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

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

---

**Action:** For information

## 9.3. 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

---

PDCO member: Brian Aylward (*ad interim*)

**Action:** For information

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

---

Draft Agenda - PCWP-HCPWP Joint meeting - 19 & 20 September 2023

Meeting Summary - PCWP HCPWP 27 and 28 June 2023

**Action:** For information

### 9.3.4. Upcoming Innovation Task Force (ITF) meetings

---

**Action:** For information

### 9.3.5. SAWP cases for PDCO

---

Scientific Advice cases with PDCO involvement in network portal

**Action:** For information

## 9.4. Cooperation within the EU regulatory network

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

---

**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

### 9.7.1. PDCO Work Plan 2023 - reporting on progress

---

PDCO Chair: Brian Aylward

**Action:** For discussion

## 9.8. Planning and reporting

### 9.8.1. EMA Business Pipeline activity and Horizon scanning

---

Q3/2023 Update of the Business Pipeline report for the human scientific committees

**Action:** For information

## 10. Any other business

### 10.1. ACT EU priority action (PA) 8 on methodology

**Action:** For information

### 10.2. Request for feedback on recommendation for inclusion of adolescents in adult trials from the Clinical Trials Coordination Group (CTCG)

PDCO member: Anette Solli Karlsen

**Action:** For adoption

## 11. Breakout sessions

### 11.1. Paediatric oncology

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

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## 11.2. Neonatology

**Action:** For discussion on Wednesday, 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.

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