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SCIENCE MEDICINES HEALTH

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Human Medicines Division

## Paediatric Committee (PDCO)

### Draft Agenda for the meeting on 19-22 July 2022

Chair: Koenraad Norga – Vice-Chair: Sabine Scherer

19 July 2022, 14:00 - 19:00, Virtual meeting

20 July 2022, 08:30 - 19:00, Virtual meeting

21 July 2022, 08:30 - 19:00, Virtual meeting

22 July 2022, 08:30 - 13:00, Virtual meeting

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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 19-22 July 2022. See July 2022 PDCO minutes (to be published post September 2022 PDCO meeting).

### 1.2. Adoption of agenda

PDCO agenda for 19-22 July 2022.

### 1.3. Adoption of the minutes

PDCO minutes for 21-24 June 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. Ex vivo expanded autologous human keratinocytes containing epidermal stem cells genetically modified with a gamma-retroviral (RV) vector expressing the full-length LAMB3 cDNA - Orphan - EMEA-003137-PIP01-21

---

Holostem Terapie Avanzate s.r.l.; Treatment of junctional epidermolysis bullosa (JEB)

Day 120 opinion

**Action:** For adoption

Dermatology

#### 2.1.2. Tezepelumab - EMEA-001613-PIP04-21

---

Treatment of chronic spontaneous urticaria

Day 120 opinion

**Action:** For adoption

Dermatology

### 2.1.3. Clazakizumab - EMEA-001371-PIP02-21

---

Treatment of chronic active antibody mediated rejection (AMR) in kidney transplantation

Day 120 opinion

**Action:** For adoption

Immunology-Rheumatology-Transplantation

### 2.1.4. Tocilizumab - EMEA-000309-PIP09-21

---

Treatment of systemic sclerosis

Day 120 opinion

**Action:** For adoption

Immunology-Rheumatology-Transplantation

### 2.1.5. Ibrexafungerp - EMEA-002535-PIP04-21

---

Treatment of invasive candidiasis

Day 120 opinion

**Action:** For adoption

Infectious Diseases

### 2.1.6. Corticotropin - EMEA-003097-PIP01-21

---

Treatment of infantile spasms

Day 120 opinion

**Action:** For adoption

Neurology

### 2.1.7. Cannabidiol - EMEA-003176-PIP01-21

---

Treatment of fragile X syndrome (FXS)

Day 120 opinion

**Action:** For adoption

Psychiatry

*Note: Withdrawal request received on 12 July 2022*

### 2.1.8. Troriluzole - EMEA-003084-PIP02-21

---

Treatment of obsessive-compulsive disorder

Day 120 opinion

**Action:** For adoption

Psychiatry

*Note: Withdrawal request received on 12 July 2022*

#### 2.1.9. Zuranolone - EMEA-003119-PIP01-21

Treatment of postpartum depression

Day 120 opinion

**Action:** For adoption

Psychiatry

#### 2.1.10. Hydrochlorothiazide / amlodipine / telmisartan - EMEA-003229-PIP01-22

Treatment of hypertension / I10 essential primary hypertension

Day 60 opinion

**Action:** For adoption

Cardiovascular Diseases

#### 2.1.11. Perindopril tert-butylamin / rosuvastatin - EMEA-003228-PIP01-22

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

Day 60 opinion

**Action:** For adoption

Cardiovascular Diseases

#### 2.1.12. Valsartan / rosuvastatin - EMEA-003240-PIP01-22

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

Day 60 opinion

**Action:** For adoption

Cardiovascular Diseases

#### 2.1.13. Tetracycline / metronidazole / bismuth - EMEA-003224-PIP01-22

Treatment of *Helicobacter pylori* infection

Day 60 opinion

**Action:** For adoption

Infectious Diseases / Gastroenterology-Hepatology

#### 2.1.14. Pegcetacoplan - EMEA-002600-PIP04-22

---

Treatment of amyotrophic lateral sclerosis

Day 60 opinion

**Action:** For adoption

Neurology

#### 2.1.15. Suvecaltamide - EMEA-003248-PIP01-22

---

Treatment of essential tremor

Day 60 opinion

**Action:** For adoption

Neurology

#### 2.1.16. Autologous CD4+ and CD8+ T cells transduced with lentiviral vector encoding a chimeric antigen receptor (CAR) directed against human B cell maturation antigen (BCMA) and preserving the T cell phenotype of the leukapheresis starting material - EMEA-003231-PIP01-22

---

Treatment of multiple myeloma

Day 60 opinion

**Action:** For adoption

Oncology

#### 2.1.17. Gallium 68-labelled prostate-specific membrane antigen-11 (68Ga-PSMA-11) - EMEA-003236-PIP01-22

---

Visualisation of prostate specific membrane antigen in prostate cancer

Day 60 opinion

**Action:** For adoption

Oncology

#### 2.1.18. Golidocitinib - EMEA-003246-PIP01-22

---

Treatment of peripheral T cell lymphoma

Day 60 opinion

**Action:** For adoption

Oncology

#### 2.1.19. Monalizumab - EMEA-002751-PIP02-22

Treatment of lung cancer

Day 60 opinion

**Action:** For adoption

Oncology

#### 2.1.20. Oleclumab - EMEA-003234-PIP01-22

Treatment of lung cancer / Treatment of pancreatic cancer

Day 60 opinion

**Action:** For adoption

Oncology

#### 2.1.21. Toripalimab - EMEA-003243-PIP01-22

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

Day 60 opinion

**Action:** For adoption

Oncology

#### 2.1.22. Vepsitamab - EMEA-003230-PIP01-22

Treatment of gastrointestinal solid tumours malignant and unspecified expressing MUC17

Day 60 opinion

**Action:** For adoption

Oncology

#### 2.1.23. Xevinapant - Orphan - EMEA-003235-PIP01-22

Merck Healthcare KGaA; Treatment of head and neck epithelial malignant neoplasms

Day 60 opinion

**Action:** For adoption

Oncology

#### 2.1.24. Dimeric protein comprised of two disulfide-linked monomers, each being a fully human fusion protein consisting of a modified extracellular domain (ECD) of the

human activin receptor type IIA (ActRIIA) fused to the fragment crystallizable (Fc) domain of human IgG1 Fc including the hinge region, CH2 and CH3 domains -  
EMA-003239-PIP01-22

---

Treatment of myelodysplastic syndrome (MDS) and myelofibrosis (MF)

Day 60 opinion

**Action:** For adoption

Oncology / Haematology-Hemostaseology

## 2.2. Opinions on Compliance Check

No item

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

### 2.3.1. Treprostinil - EMA-000207-PIP01-08-M07

---

Ferrer Internacional, S.A.; Treatment of pulmonary arterial hypertension

Day 60 opinion

**Action:** For adoption

Cardiovascular Diseases

### 2.3.2. Evinacumab - EMA-002298-PIP01-17-M04

---

Regeneron Ireland DAC; Treatment of elevated cholesterol

Day 60 opinion

**Action:** For adoption

Endocrinology-Gynaecology-Fertility-Metabolism

### 2.3.3. Guselkumab - EMA-001523-PIP04-19-M01

---

Janssen-Cilag International N.V.; Treatment of ulcerative colitis: ICD K51

Day 60 opinion

**Action:** For adoption

Gastroenterology-Hepatology

### 2.3.4. Odevixibat - Orphan - EMA-002054-PIP03-20-M01

---

Albireo AB; Treatment of Alagille syndrome

Day 60 opinion

**Action:** For adoption

Gastroenterology-Hepatology

### 2.3.5. Ustekinumab - EMEA-000311-PIP04-13-M05

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

Day 60 opinion

**Action:** For adoption

Gastroenterology-Hepatology

### 2.3.6. Plasma kallikrein inhibitor - EMEA-002723-PIP01-19-M01

KalVista Pharmaceuticals Ltd; Treatment of hereditary angioedema

Day 60 opinion

**Action:** For adoption

Haematology-Hemostaseology

### 2.3.7. Roxadustat - EMEA-001557-PIP01-13-M06

Astellas Pharma Europe B.V.; Treatment of anaemia due to chronic disorders

Day 60 opinion

**Action:** For adoption

Haematology-Hemostaseology

### 2.3.8. Vonicog alfa - EMEA-001164-PIP01-11-M06

Baxalta Innovations GmbH; Treatment of Von Willebrand disease

Day 60 opinion

**Action:** For adoption

Haematology-Hemostaseology

### 2.3.9. Apremilast - EMEA-000715-PIP02-11-M06

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

Day 60 opinion

**Action:** For adoption

Immunology-Rheumatology-Transplantation

### 2.3.10. Apremilast - EMEA-000715-PIP05-13-M05

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

Day 60 opinion

**Action:** For adoption

Immunology-Rheumatology-Transplantation

### 2.3.11. Baricitinib - EMEA-001220-PIP07-20-M01

Eli Lilly and Company Limited; Treatment of coronavirus disease 2019 (COVID-19)

Day 60 opinion

**Action:** For adoption

Immunology-Rheumatology-Transplantation

### 2.3.12. Belimumab - EMEA-000520-PIP02-13-M04

Glaxo Group Limited; Treatment of systemic lupus erythematosus

Day 60 opinion

**Action:** For adoption

Immunology-Rheumatology-Transplantation

### 2.3.13. Griselimab setaritox / dafsolimab setaritox - Orphan - EMEA-002087-PIP01-16-M01

Xenikos B.V.; Treatment of acute graft versus host disease

Day 60 opinion

**Action:** For adoption

Immunology-Rheumatology-Transplantation

### 2.3.14. Upadacitinib - EMEA-001741-PIP04-17-M03

AbbVie Ltd; Treatment of atopic dermatitis

Day 60 opinion

**Action:** For adoption

Immunology-Rheumatology-Transplantation / Dermatology

### 2.3.15. Baloxavir marboxil - EMEA-002440-PIP01-18-M03

Roche Registration GmbH; Prevention of influenza / Treatment of influenza

Day 60 opinion



**Action:** For adoption

Infectious Diseases

#### [2.3.16. BNT162b2 / tozinameran - EMEA-002861-PIP02-20-M04](#)

---

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

Day 60 opinion

**Action:** For adoption

Infectious Diseases

#### [2.3.17. Cabotegravir - EMEA-001418-PIP01-13-M05](#)

---

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

Day 60 opinion

**Action:** For adoption

Infectious Diseases

#### [2.3.18. Cobicistat - EMEA-000969-PIP01-10-M06](#)

---

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

Day 60 opinion

**Action:** For adoption

Infectious Diseases

#### [2.3.19. Emtricitabine / tenofovir alafenamide - EMEA-001577-PIP02-14-M05](#)

---

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

Day 60 opinion

**Action:** For adoption

Infectious Diseases

#### [2.3.20. Lamivudine / dolutegravir - EMEA-001940-PIP01-16-M04](#)

---

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

Day 60 opinion

**Action:** For adoption

Infectious Diseases

### 2.3.21. Rilpivirine - EMEA-000317-PIP02-18-M01

---

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

Day 60 opinion

**Action:** For adoption

Infectious Diseases

### 2.3.22. Tenofovir alafenamide / rilpivirine / emtricitabine - EMEA-001679-PIP01-14-M02

---

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

Day 60 opinion

**Action:** For adoption

Infectious Diseases

### 2.3.23. Zoliflodacin - EMEA-002599-PIP01-19-M01

---

Entasis Therapeutic Inc.; Treatment of gonococcal infection

Day 60 opinion

**Action:** For adoption

Infectious Diseases

### 2.3.24. Fenfluramine hydrochloride - Orphan - EMEA-001990-PIP01-16-M05

---

Zogenix International Ltd; Treatment of Dravet syndrome

Day 60 opinion

**Action:** For adoption

Neurology

### 2.3.25. Ozanimod (hydrochloride) - EMEA-001710-PIP02-14-M07

---

Bristol-Myers Squibb Pharma EEIG; Treatment of multiple sclerosis

Day 60 opinion

**Action:** For adoption

Neurology

### 2.3.26. Peginterferon beta-1a - EMEA-001129-PIP01-11-M05

---

Biogen Idec Ltd; Treatment of multiple sclerosis

Day 60 opinion

**Action:** For adoption

Neurology

---

#### 2.3.27. Cabozantinib - Orphan - EMEA-001143-PIP01-11-M05

---

Ipsen Pharma; Treatment of malignant solid tumours

Day 60 opinion

**Action:** For adoption

Oncology

---

#### 2.3.28. Copanlisib - Orphan - EMEA-001757-PIP02-15-M03

---

Bayer AG; Treatment of mature B cell neoplasms / Treatment of all conditions included in the category of malignant neoplasms (except haematopoietic and lymphoid tissue)

Day 60 opinion

**Action:** For adoption

Oncology

---

#### 2.3.29. Durvalumab - EMEA-002028-PIP01-16-M03

---

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

Day 60 opinion

**Action:** For adoption

Oncology

---

#### 2.3.30. Tremelimumab - Orphan - EMEA-002029-PIP01-16-M03

---

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

Day 60 opinion

**Action:** For adoption

Oncology

---

#### 2.3.31. Inotuzumab ozogamicin - Orphan - EMEA-001429-PIP01-13-M06

---

Pfizer Europe MA EEIG; Treatment of acute lymphoblastic leukaemia

Day 60 opinion

**Action:** For adoption

Oncology / Haematology-Hemostaseology

---

### 2.3.32. Lomitapide (as lomitapide mesylate) - EMEA-001124-PIP01-10-M05

---

Amryt Pharmaceuticals DAC; Treatment of heterozygous and homozygous familial hypercholesterolaemia

Day 60 opinion

**Action:** For adoption

Other

---

### 2.3.33. Cannabidiol / delta-9-tetrahydrocannabinol - EMEA-000181-PIP02-13-M01

---

GW Pharma (International) B.V; Treatment of pain

Day 60 opinion

**Action:** For adoption

Pain

---

### 2.3.34. Adrenaline (epinephrine) - EMEA-002749-PIP01-19-M02

---

ARS Pharmaceuticals IRL, Limited; Treatment of allergic reactions

Day 60 opinion

**Action:** For adoption

Pneumology - Allergology

---

### 2.3.35. Cholera vaccine, recombinant, live, oral (strain CVD 103-HgR) - EMEA-001490-PIP01-13-M03

---

Emergent Netherlands B.V.; Treatment of cholera disease caused by *Vibrio cholerae* serogroup O1

Day 60 opinion

**Action:** For adoption

Vaccines

## 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. Asciminib - EMEA-C2-002347-PIP01-18

---

Novartis Europharm Limited; Treatment of chronic myeloid leukaemia

Day 30 letter

**Action:** For information

Oncology / Haematology-Hemostaseology

### 2.7.2. Leriglitazone - EMEA-C1-002106-PIP01-16-M02

---

Minoryx Therapeutics S.L.; Treatment of adrenoleukodystrophy

Day 30 letter

**Action:** For information

Neurology

### 2.7.3. Upadacitinib - EMEA-C1-001741-PIP03-16-M02

---

AbbVie Ltd; Treatment of Crohn's disease

Day 30 letter

**Action:** For information

Gastroenterology-Hepatology

### 2.7.4. Ruxolitinib (phosphate) - EMEA-C2-000901-PIP03-16-M02

---

Novartis Europharm Limited; Treatment of acute graft versus host disease (aGVHD)

Day 30 letter

**Action:** For information

Oncology

### 2.7.5. Ruxolitinib (phosphate) - EMEA-C2-000901-PIP04-17-M02

---

Novartis Europharm Limited; Treatment of chronic graft versus host disease

Day 30 letter

**Action:** For information

Oncology

### 2.7.6. Modified Vaccinia Ankara - Bavarian Nordic virus (smallpox) - EMEA-C1-001161-PIP02-11-M01

---

Bavarian Nordic A/S; Prevention of smallpox, monkeypox and related orthopoxvirus infection

Day 30 letter

**Action:** For information

Vaccines

### 2.7.7. Fluticasone furoate / triphenylacetic acid - 4-{{(1R)-2-[(6-{{2-[(2,6-dichlorobenzyl)oxy]ethoxy}hexyl)amino]-1-hydroxyethyl}}-2-(hydroxymethyl)phenol - EMEA-C4-000431-PIP01-08-M12

---

Glaxo Group Limited; Treatment of asthma

Day 30 letter

**Action:** For information

Pneumology – Allergology

## 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. Treprostinil - EMEA-003182-PIP01-22

---

Treatment of pulmonary arterial hypertension

Day 90 discussion

**Action:** For discussion

Cardiovascular Diseases

### 3.1.2. Beremagene geperpavec - Orphan - EMEA-002472-PIP03-22

---

Krystal Biotech, Inc.; Treatment of dystrophic epidermolysis bullosa

Day 90 discussion

**Action:** For discussion

Dermatology

### 3.1.3. Recombinant fusion protein linking iduronate 2-sulfatase to engineered Fc with binding site for transferrin receptor - EMEA-002845-PIP01-20

---

Treatment of mucopolysaccharidosis II (Hunter syndrome)

Day 90 discussion

**Action:** For discussion

Endocrinology-Gynaecology-Fertility-Metabolism

### 3.1.4. Resmetirom - EMEA-003087-PIP01-21

---

Treatment of non-alcoholic steatohepatitis

Day 90 discussion

**Action:** For discussion

Gastroenterology-Hepatology

### 3.1.5. Cenerimod - EMEA-003108-PIP01-21

---

Treatment of systemic lupus erythematosus (SLE)

Day 90 discussion

**Action:** For discussion

Immunology-Rheumatology-Transplantation

### 3.1.6. Efavaleukin alfa - EMEA-003156-PIP01-21

---

Treatment of systemic lupus erythematosus (SLE)

Day 90 discussion

**Action:** For discussion

Immunology-Rheumatology-Transplantation

### 3.1.7. Ianalumab - EMEA-002338-PIP03-21

---

Treatment of systemic lupus erythematosus (SLE)

Day 90 discussion

**Action:** For discussion

Immunology-Rheumatology-Transplantation

### 3.1.8. Branaplam - EMEA-002204-PIP02-20

---

Treatment of Huntington's disease

Day 90 discussion

**Action:** For discussion

Neurology

### 3.1.9. Satralizumab - Orphan - EMEA-001625-PIP03-21

---

Roche Registration GmbH; Treatment of myelin oligodendrocyte glycoprotein antibody-associated disease

Day 90 discussion

**Action:** For discussion

Neurology

### 3.1.10. Odronextamab - EMEA-003149-PIP01-21

---

Aggressive mature B cell non-Hodgkin lymphoma (B-NHL)

Day 90 discussion

**Action:** For discussion

Oncology

### 3.1.11. Freeze-dried allergen extract of *Betula pendula* pollen - EMEA-003117-PIP02-21

---

Diagnosis of IgE mediated allergy to tree pollen of the birch group

Day 90 discussion

**Action:** For discussion

Pneumology - Allergology

### 3.1.12. Fusion protein composed of the first 2 immunoglobulin (Ig)-like domains of the human ROBO2 fused to human IgG1 Fc (PF-06730512) - Orphan - EMEA-003157-PIP01-21

---

Pfizer Europe MA EEIG; Treatment of focal segmental glomerulosclerosis (FSGS)

Day 90 discussion

**Action:** For discussion

Uro-nephrology



### 3.1.13. Yellow fever virus, strain vYF-247 - EMEA-003030-PIP02-21

---

Prevention of yellow fever disease

Day 90 discussion

**Action:** For discussion

Vaccines

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

---

Treatment of atopic dermatitis

Day 60 discussion

**Action:** For discussion

Dermatology

### 3.1.15. CRN04777 - Orphan - EMEA-003242-PIP01-22

---

Crinetics Pharmaceuticals, Inc.; Treatment of congenital hyperinsulinism

Day 60 discussion

**Action:** For discussion

Endocrinology-Gynaecology-Fertility-Metabolism

### 3.1.16. EMEA-003241-PIP01-22

---

Treatment of sickle cell disease

Day 60 discussion

**Action:** For discussion

Haematology-Hemostaseology

### 3.1.17. Itolizumab - Orphan - EMEA-003208-PIP02-22

---

Biocon Pharma Malta-I Limited; Treatment of acute graft versus host disease

Day 60 discussion

**Action:** For discussion

Immunology-Rheumatology-Transplantation

### 3.1.18. Cilgavimab / tixagevimab - EMEA-003079-PIP01-22

---

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

Day 60 discussion

**Action:** For discussion

Infectious Diseases

### 3.1.19. [Opelconazole - EMEA-003249-PIP01-22](#)

---

Treatment of bronchopulmonary aspergillosis

Day 60 discussion

**Action:** For discussion

Infectious Diseases

### 3.1.20. [Enibarcimab - EMEA-003244-PIP01-22](#)

---

Treatment of sepsis

Day 60 discussion

**Action:** For discussion

Neonatology - Paediatric Intensive Care / Cardiovascular Diseases

### 3.1.21. [Cemdisiran sodium - Orphan - EMEA-003237-PIP01-22](#)

---

Regeneron Ireland DAC; Treatment of generalised myasthenia gravis (gMG)

Day 60 discussion

**Action:** For discussion

Neurology

### 3.1.22. [Pozelimab - EMEA-003238-PIP01-22](#)

---

Treatment of generalised myasthenia gravis (gMG)

Day 60 discussion

**Action:** For discussion

Neurology

### 3.1.23. [Lutetium \(177Lu\) edotreotide - Orphan - EMEA-003245-PIP01-22](#)

---

ITM Solucin GmbH; Treatment of somatostatin receptor (SSTR)-positive tumours

Day 60 discussion

**Action:** For discussion

Oncology

### 3.1.24. Obinutuzumab - Orphan - EMEA-001207-PIP04-22

---

Roche Registration GmbH; Prevention of cytokine release syndrome

Day 60 discussion

**Action:** For discussion

Oncology

### 3.1.25. Tinalarebant - Orphan - EMEA-003225-PIP01-22

---

Belite Bio, Inc; Treatment of Stargardt disease

Day 60 discussion

**Action:** For discussion

Ophthalmology

### 3.1.26. Tirzepatide - EMEA-002360-PIP02-22

---

Treatment of obesity

Day 60 discussion

**Action:** For discussion

Other

### 3.1.27. 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 60 discussion

**Action:** For discussion

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

### 3.1.28. ABNCoV2 (AV2-cVLP-RBD SARS-CoV-2) - EMEA-003184-PIP01-22

---

Prevention of coronavirus disease 2019 (COVID-19)

Day 60 discussion

**Action:** For discussion

Vaccines / Infectious Diseases

### 3.1.29. Rosuvastatin / telmisartan - EMEA-003262-PIP01-22

---

Treatment of hypertension / Prevention of cardiovascular events

Day 30 discussion

**Action:** For discussion

Cardiovascular Diseases

### 3.1.30. [Adapalene, micronised / benzoyl peroxide, hydrous / clindamycin - EMEA-003263-PIP01-22](#)

---

Treatment of acne vulgaris

Day 30 discussion

**Action:** For discussion

Dermatology

### 3.1.31. [Fluorine \(18F\) PSMA-1007 - EMEA-003250-PIP01-22](#)

---

For the diagnosis of prostate cancer recurrence after previous definitive treatment

Day 30 discussion

**Action:** For discussion

Diagnostic

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

---

Genzyme Europe B.V.; Treatment of achondroplasia

Day 30 discussion

**Action:** For discussion

Endocrinology-Gynaecology-Fertility-Metabolism

### 3.1.33. [Crovalimab - EMEA-002709-PIP03-22](#)

---

Treatment of Guillain-Barré syndrome (GBS)

Day 30 discussion

**Action:** For discussion

Haematology-Hemostaseology

### 3.1.34. [Luspatercept - Orphan - EMEA-001521-PIP03-22](#)

---

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

Day 30 discussion

**Action:** For discussion

Haematology-Hemostaseology

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

---

Roche Registration GmbH; Treatment of glomerulonephritis and nephrotic syndrome

Day 30 discussion

**Action:** For discussion

Immunology-Rheumatology-Transplantation

### 3.1.36. [EMEA-003252-PIP01-22](#)

---

Treatment of multiple system atrophy

Day 30 discussion

**Action:** For discussion

Neurology

### 3.1.37. [Pridopidine HCl - Orphan - EMEA-003174-PIP02-22](#)

---

Prilenia Therapeutics B.V.; Treatment of amyotrophic lateral sclerosis (ALS)

Day 30 discussion

**Action:** For discussion

Neurology

### 3.1.38. [EMEA-003260-PIP01-22](#)

---

Treatment of dedifferentiated liposarcoma

Day 30 discussion

**Action:** For discussion

Oncology

### 3.1.39. [EMEA-003197-PIP02-22](#)

---

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

Day 30 discussion

**Action:** For discussion

Oncology

### 3.1.40. Trilaciclib - EMEA-002534-PIP03-22

---

Treatment of breast malignant neoplasms

Day 30 discussion

**Action:** For discussion

Oncology

### 3.1.41. Valemetostat tosilate - Orphan - EMEA-003256-PIP01-22

---

Daiichi Sankyo Europe GmbH; Treatment of mature T cell neoplasms

Day 30 discussion

**Action:** For discussion

Oncology

### 3.1.42. Zilovetamab 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 30 discussion

**Action:** For discussion

Oncology

### 3.1.43. Batoclimab - EMEA-003162-PIP02-22

---

Treatment of thyroid eye disease (TED)

Day 30 discussion

**Action:** For discussion

Ophthalmology

### 3.1.44. EMEA-003258-PIP01-22

---

Treatment of obesity

Day 30 discussion

**Action:** For discussion

Other

### 3.1.45. Tadalafil / finasteride - EMEA-003261-PIP01-22

---

Treatment of the signs and symptoms of benign prostatic hyperplasia (BPH) in adult males

---

with an enlarged prostate

Day 30 discussion

**Action:** For discussion

Other

### 3.1.46. Lidocaine - EMEA-003255-PIP01-22

---

Prevention of pain of the oral mucosa, prior to injection of a local anaesthetic agent for dental procedures

Day 30 discussion

**Action:** For discussion

Pain

### 3.1.47. EMEA-003254-PIP01-22

---

Prevention of pulmonary infection/colonisation by improvement in airway mucosal clearance in patients with cystic fibrosis

Day 30 discussion

**Action:** For discussion

Pneumology - Allergology

### 3.1.48. Aticaprant - EMEA-003251-PIP01-22

---

Treatment of major depressive disorder

Day 30 discussion

**Action:** For discussion

Psychiatry

### 3.1.49. Human papillomavirus type 58 L1 protein / human papillomavirus type 52 L1 protein / human papillomavirus type 45 L1 protein / human papillomavirus type 33 L1 protein / human papillomavirus type 31 L1 protein / human papillomavirus type 18 L1 protein / human papillomavirus type 16 L1 protein / human papillomavirus type 11 L1 protein / human papillomavirus type 6 L1 protein - EMEA-003209-PIP01-22

---

Prevention of infection by human papillomavirus (HPV)

Day 30 discussion

**Action:** For discussion

Vaccines / Infectious Diseases

## 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. Edoxaban tosilate - EMEA-C4-000788-PIP02-11-M11

---

Daiichi-Sankyo Europe GmbH; Treatment of venous thromboembolism

Day 30 discussion

**Action:** For discussion

Cardiovascular Diseases

### 3.2.2. Semaglutide - EMEA-C2-001441-PIP03-17-M02

---

Novo Nordisk A/S; Treatment of obesity

Day 30 discussion

**Action:** For discussion

Endocrinology-Gynaecology-Fertility-Metabolism

### 3.2.3. Artesunate - EMEA-C-002710-PIP01-19

---

Amivas Ireland Limited; Treatment of malaria

Day 30 discussion

**Action:** For discussion

Infectious Diseases

### 3.2.4. Lanadelumab - EMEA-C-001864-PIP01-15-M07

---

Takeda Pharmaceuticals International AG Ireland Branch; Prevention of hereditary angioedema attacks

Day 30 discussion

**Action:** For discussion

Other

### 3.2.5. Nintedanib esylate - EMEA-C-001006-PIP05-18-M01

---

Boehringer Ingelheim International GmbH; Treatment of fibrosing interstitial lung diseases

Day 30 discussion

**Action:** For discussion

Pneumology - Allergology



3.2.6. Influenza virus A / turkey / turkey / 1 / 2005 (H5N1) NIBRG-23 strain, HA surface antigen - EMEA-C-002869-PIP03-21

---

Seqirus Netherlands B.V.; Influenza due to identified zoonotic or pandemic influenza virus

Day 30 discussion

**Action:** For discussion

Vaccines

3.2.7. Influenza virus surface antigens (haemagglutinin and neuraminidase) of strain A/H5N1 - EMEA-C1-002869-PIP01-21

---

Seqirus Netherlands B.V.; Influenza due to identified zoonotic or pandemic influenza virus

Day 30 discussion

**Action:** For discussion

Vaccines

3.2.8. Recombinant influenza hemagglutinin-strain B (Victoria lineage) / recombinant influenza hemagglutinin-strain A (H1N1 subtype) / recombinant influenza hemagglutinin-strain A (H3N2 subtype) / recombinant influenza hemagglutinin-strain B (Yamagata lineage) - EMEA-C3-002418-PIP01-18-M02

---

Sanofi Pasteur; Prevention of influenza infection

Day 30 discussion

**Action:** For discussion

Vaccines

3.2.9. Recombinant vesicular stomatitis virus - Zaire Ebolavirus vaccine (live) - EMEA-C1-001786-PIP01-15-M02

---

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

Day 30 discussion

**Action:** For discussion

Vaccines

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

3.3.1. Ambrisentan - Orphan - EMEA-000434-PIP01-08-M09

---

Glaxo Group Limited; Treatment of pulmonary arterial hypertension

Day 30 discussion

**Action:** For discussion  
Cardiovascular Diseases

### 3.3.2. Finerenone - EMEA-001623-PIP03-20-M01

---

Bayer AG; Treatment of heart failure  
Day 30 discussion  
**Action:** For discussion  
Cardiovascular Diseases

### 3.3.3. Sotatercept - Orphan - EMEA-002756-PIP01-19-M01

---

Merck Sharp & Dohme B.V.; Treatment of pulmonary arterial hypertension  
Day 30 discussion  
**Action:** For discussion  
Cardiovascular Diseases

### 3.3.4. Vericiguat - EMEA-001636-PIP01-14-M03

---

Bayer AG; Treatment of left ventricular failure  
Day 30 discussion  
**Action:** For discussion  
Cardiovascular Diseases

### 3.3.5. Ritlecitinib - EMEA-002451-PIP01-18-M01

---

Pfizer Europe MA EEIG; Treatment of alopecia areata  
Day 30 discussion  
**Action:** For discussion  
Dermatology

### 3.3.6. Gadopiclenol - EMEA-001949-PIP02-18-M02

---

Guerbet; Diagnostic  
Day 30 discussion  
**Action:** For discussion  
Diagnostic

3.3.7. [3,6-diamino-2,5-bis{N-\[\(1R\)-1-carboxy-2-hydroxyethyl\]carbamoyl}pyrazine \(designated MB-102\) - EMEA-001983-PIP01-16-M01](#)

---

MediBeacon Inc.; Monitoring of renal function

Day 30 discussion

**Action:** For discussion

Diagnostic / Uro-nephrology

3.3.8. [Levonorgestrel - EMEA-002474-PIP02-18-M01](#)

---

Chemo Research, S.L.; Contraception

Day 30 discussion

**Action:** For discussion

Endocrinology-Gynaecology-Fertility-Metabolism

3.3.9. [Etrasimod L-arginine - EMEA-002713-PIP01-19-M02](#)

---

Arena Pharmaceuticals, Inc.; Treatment of ulcerative colitis

Day 30 discussion

**Action:** For discussion

Gastroenterology-Hepatology

3.3.10. [Golimumab - EMEA-000265-PIP02-11-M04](#)

---

Janssen Biologics B.V.; Treatment of ulcerative colitis

Day 30 discussion

**Action:** For discussion

Gastroenterology-Hepatology

3.3.11. [Insulin human - EMEA-002116-PIP01-17-M01](#)

---

ELGAN Pharma Ltd; Treatment of intestinal malabsorption in preterm infants

Day 30 discussion

**Action:** For discussion

Gastroenterology-Hepatology

3.3.12. [Maralixibat chloride - Orphan - EMEA-001475-PIP03-17-M03](#)

---

Mirum Pharmaceuticals; Treatment of progressive familial intrahepatic cholestasis

Day 30 discussion

**Action:** For discussion

Gastroenterology-Hepatology

### 3.3.13. Tofacitinib - EMEA-000576-PIP03-12-M06

---

Pfizer Europe MA EEIG; Treatment of ulcerative colitis

Day 30 discussion

**Action:** For discussion

Gastroenterology-Hepatology

### 3.3.14. Efanesoctocog alfa - Orphan - EMEA-002501-PIP01-18-M03

---

Swedish Orphan Biovitrum AB (Publ).; Treatment of haemophilia A

Day 30 discussion

**Action:** For discussion

Haematology-Hemostaseology

### 3.3.15. Etranacogene dezaparovec - Orphan - EMEA-002722-PIP01-19-M02

---

CSL Behring GmbH; Treatment of haemophilia B

Day 30 discussion

**Action:** For discussion

Haematology-Hemostaseology

### 3.3.16. Marstacimab - Orphan - EMEA-002285-PIP02-19-M02

---

Pfizer Europe MAA EEIG; Treatment of congenital haemophilia B / Treatment of congenital haemophilia A

Day 30 discussion

**Action:** For discussion

Haematology-Hemostaseology

### 3.3.17. Guselkumab - EMEA-001523-PIP03-18-M02

---

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

### 3.3.18. Secukinumab - EMEA-000380-PIP06-19-M01

---

Novartis Europharm Limited; Treatment of systemic lupus erythematosus

Day 30 discussion

**Action:** For discussion

Immunology-Rheumatology-Transplantation

### 3.3.19. Cefiderocol - EMEA-002133-PIP01-17-M03

---

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

Day 30 discussion

**Action:** For discussion

Infectious Diseases

### 3.3.20. Gepotidacin - EMEA-002443-PIP01-18-M01

---

GlaxoSmithKline Trading Services Limited; Treatment of uncomplicated urinary tract infections (uUTI)

Day 30 discussion

**Action:** For discussion

Infectious Diseases

### 3.3.21. Gepotidacin - EMEA-002443-PIP02-18-M01

---

GlaxoSmithKline Trading Services Limited; Treatment of uncomplicated urogenital gonorrhoea (GC)

Day 30 discussion

**Action:** For discussion

Infectious Diseases

### 3.3.22. Maribavir - Orphan - EMEA-000353-PIP02-16-M02

---

Takeda Pharmaceuticals International AG Ireland Branch; Treatment of cytomegalovirus (CMV) infection

Day 30 discussion

**Action:** For discussion

Infectious Diseases

### 3.3.23. Taniborbactam / cefepime - EMEA-002576-PIP01-19-M01

---

Venatorx Pharmaceuticals, Inc.; Treatment of gram-negative bacterial infections

Day 30 discussion

**Action:** For discussion

Infectious Diseases

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### 3.3.24. [D-Sorbitol / naltrexone HCl / \(RS\)-baclofen - Orphan - EMEA-002164-PIP01-17-M03](#)

Pharnext SA; Charcot-Marie-Tooth disease type 1A

Day 30 discussion

**Action:** For discussion

Neurology

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### 3.3.25. [Diroximel fumarate - EMEA-002685-PIP02-19-M01](#)

Biogen Netherlands B.V; Treatment of multiple sclerosis

Day 30 discussion

**Action:** For discussion

Neurology

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### 3.3.26. [Ocrelizumab - EMEA-000310-PIP03-10-M06](#)

Roche Registration GmbH; Treatment of multiple sclerosis

Day 30 discussion

**Action:** For discussion

Neurology

---

### 3.3.27. [Rozanolixizumab - Orphan - EMEA-002681-PIP01-19-M01](#)

UCB Pharma S.A.; Treatment of myasthenia gravis

Day 30 discussion

**Action:** For discussion

Neurology

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### 3.3.28. [Enasidenib - Orphan - EMEA-001798-PIP02-16-M01](#)

Bristol-Myers Squibb Pharma EEIG; Treatment of acute myeloid leukaemia

Day 30 discussion

**Action:** For discussion

Oncology / Haematology-Hemostaseology

### 3.3.29. Atropine sulphate - EMEA-002545-PIP01-19-M01

---

Fondazione Per La Ricerca Farmacologica Gianni Benzi Onlus; Treatment of myopia

Day 30 discussion

**Action:** For discussion

Ophthalmology

### 3.3.30. Alpelisib - Orphan - EMEA-002016-PIP03-19-M02

---

Novartis Europharm Limited; Treatment of PIK3CA related overgrowth spectrum

Day 30 discussion

**Action:** For discussion

Other

### 3.3.31. Atidarsagene autotemcel - Orphan - EMEA-001765-PIP02-15-M04

---

Orchard Therapeutics (Netherlands) B.V.; Treatment of metachromatic leukodystrophy

Day 30 discussion

**Action:** For discussion

Other

### 3.3.32. Burosumab: Human recombinant IgG1 monoclonal antibody to fibroblast growth factor 23 (FGF23); KRN23 - Orphan - EMEA-001659-PIP01-15-M06

---

Kyowa Kirin Holdings B.V.; Treatment of X-linked hypophosphataemia

Day 30 discussion

**Action:** For discussion

Other

### 3.3.33. Eliglustat - Orphan - EMEA-000461-PIP02-11-M05

---

Genzyme Europe B.V.; Treatment of Gaucher disease ( ICD-9-CM Diagnosis 272.7, Lipidoses) type 1 / Treatment of Gaucher disease (ICD-9-CM Diagnosis 272.7, Lipidoses) type 3

Day 30 discussion

**Action:** For discussion

Other

### 3.3.34. Begelomab - Orphan - EMEA-001744-PIP01-14-M01

---

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

Day 30 discussion

**Action:** For discussion

Other / Immunology-Rheumatology-Transplantation

---

### 3.3.35. Bupivacaine - EMEA-000877-PIP03-17-M04

---

Pacira Ltd; Postsurgical analgesia

Day 30 discussion

**Action:** For discussion

Pain

---

### 3.3.36. Finerenone - EMEA-001623-PIP01-14-M05

---

Bayer AG; Treatment of chronic kidney disease

Day 30 discussion

**Action:** For discussion

Uro-nephrology

---

### 3.3.37. Meningococcal group Y oligosaccharides conjugated to *Corynebacterium diphtheriae* CRM197 protein / recombinant *Neisseria meningitidis* group B Protein 961c / recombinant *Neisseria meningitidis* group B Protein 287- 953 / meningococcal group C oligosaccharides conjugated to *Corynebacterium diphtheriae* CRM197 protein / meningococcal group A oligosaccharides conjugated to *Corynebacterium diphtheriae* CRM197 protein / meningococcal group W-135 oligosaccharides conjugated to *Corynebacterium diphtheriae* CRM197 protein / recombinant *Neisseria meningitidis* group B Protein 936-741 / outer membrane vesicles (OMV) from *N. meningitidis* strain NZ 98/254 - EMEA-001260-PIP01-11-M02

---

GlaxoSmithKline Biologicals SA; Prevention of meningococcal meningitis

Day 30 discussion

**Action:** For discussion

Vaccines

---

### 3.3.38. Ad26.RSV.preF - EMEA-002172-PIP02-17-M02

---

Janssen-Cilag International NV; Prevention of lower respiratory tract disease (LRTD) caused by respiratory syncytial virus (RSV)

Day 30 discussion

**Action:** For discussion

Vaccines / Infectious Diseases



## 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 16 August 2022 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. Adeno-associated viral vector serotype 2 expressing sCD59 (JNJ-81201887) – EMEA-02-2022

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Janssen-Cilag International N.V; All classes of medicinal products for treatment of age-related macular degeneration and diabetic macular oedema / Treatment of geographic atrophy (GA) secondary to dry age-related macular degeneration (AMD)

**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

#### 7.1.1. Pyrrol-hydroxyethylpyridin-3-ol derivative (MIJ821) - EMEA-002946-PIP01-20

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Novartis Europharm Limited

The request for the PDCO is to confirm whether Treatment Resistant Depression (TRD) is covered by the condition major depressive disorder (MDD) in the PIP.

**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

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**Action:** For information

#### 9.1.3. Election of PDCO Chairperson

---

**Action:** For adoption

#### 9.1.4. Strategic Review and Learning Meeting (SRLM) – Prague, 6 – 7 October 2022

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PDCO member: Tomáš Boráň

**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

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PDCO member: Brian Aylward

**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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**Action:** For information

### 9.4.2. C4C Multistakeholder meeting on type I diabetes

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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

No item

## 10. Any other business

### 10.1. COVID-19 update

**Action:** For information

### 10.2. COVID-19 disease incidence in paediatric populations study

**Action:** For information

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

PDCO members: Carine de Beaufort, Agnes Gyurasics; CHMP member: Kristina Dunder

**Action:** For adoption

### 10.4. EMA records management system – update on Sharepoint migration

**Action:** For discussion

### 10.5. Exparel liposomal - EMEA/H/C/004586/II/0005 PDCO consultation from CHMP

**Action:** For discussion

### 10.6. ICH E11A case example publication

PDCO member: Kristin Karlsson

**Action:** For discussion

### 10.7. Real World Evidence update, including DARWIN EU®

**Action:** For information

## 11. Breakout sessions

### 11.1. Internal PDCO Operations

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

## **11.2. Neonatology**

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

## **11.3. Paediatric oncology**

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

## **11.4. 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/)