



EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

21 February 2023
EMA/PDCO/49245/2023
Human Medicines Division

Paediatric Committee (PDCO)

Draft Agenda for the meeting on 21-24 February 2023

Chair: Brian Aylward – Vice-Chair: Sylvie Benchetrit

21 February 2023, 14:00- 19:30, room 2C

22 February 2023, 08:30- 19:30, room 2C

23 February 2023, 08:30- 19:30, room 2C

24 February 2023, 08:30- 13:00, room 2C

Health and safety information

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

Disclaimers

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

Note on access to documents

Some documents mentioned in the agenda cannot be released at present following a request for access to documents within the framework of Regulation (EC) No 1049/2001 as they are subject to ongoing procedures for which a final decision has not yet been adopted. They will become public when adopted or considered public according to the principles stated in the Agency policy on access to documents (EMA/127362/2006).



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1. Introductions

1.1. Welcome and declarations of interest of members, alternates and experts

Pre-meeting list of participants and restrictions in relation to declarations of interests applicable to the items of the agenda for the PDCO plenary session to be held 21-24 February 2023. See February 2023 PDCO minutes (to be published post March 2023 PDCO meeting).

1.2. Adoption of agenda

PDCO agenda for 21-24 February 2023.

1.3. Adoption of the minutes

PDCO minutes for 17-20 January 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. Pudexacianinium - EMEA-003099-PIP01-21

Visualisation of ureter

Day 120 opinion

Action: For adoption

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

2.1.2. Cilgavimab / tixagevimab - EMEA-003079-PIP01-22

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

Day 120 opinion

Action: For adoption

Infectious Diseases

2.1.3. Vilobelimab - EMEA-003080-PIP03-22

Treatment of severe coronavirus disease 2019 (COVID-19)

Day 120 opinion

Action: For adoption

Infectious Diseases

2.1.4. Zilovetamab vedotin - Orphan - EMEA-003257-PIP01-22

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

Day 120 opinion

Action: For adoption

Oncology

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

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

Day 120 opinion

Action: For adoption

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

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

Prevention of coronavirus disease 2019 (COVID-19)

Day 120 opinion

Action: For adoption

Vaccines / Infectious Diseases

2.1.7. Efzofitimod - EMEA-003352-PIP01-22

Treatment of sarcoidosis

Day 60 opinion

Action: For adoption

Immunology-Rheumatology-Transplantation

2.1.8. Utreloxastat - EMEA-003369-PIP01-22

Treatment of amyotrophic lateral sclerosis

Day 60 opinion

Action: For adoption

Neurology

2.1.9. EMEA-003358-PIP01-22

Treatment of breast malignant neoplasms

Day 60 opinion

Action: For adoption

Oncology

2.1.10. Lutetium 177-labelled radiohybrid prostate-specific membrane antigen-10.1 ((177Lu) rhPSMA-10.1 - EMEA-003353-PIP01-22

Treatment of PSMA-expressing prostate cancer

Day 60 opinion

Action: For adoption

Oncology

2.1.11. Acoltremon - EMEA-003351-PIP01-22

Treatment of dry eye disease

Day 60 opinion

Action: For adoption

Ophthalmology

2.1.12. Ciclosporin - EMEA-003366-PIP01-22

Treatment of dry eye disease (DED)

Day 60 opinion

Action: For adoption

Ophthalmology

2.1.13. Meningococcal group Y oligosaccharide conjugated to *Corynebacterium diphtheriae* CRM197 protein / meningococcal group W-135 oligosaccharide conjugated to *Corynebacterium diphtheriae* CRM197 protein / meningococcal group C oligosaccharide conjugated to *Corynebacterium diphtheriae* CRM197 protein /

meningococcal group A oligosaccharide conjugated to *Corynebacterium diphtheriae* CRM197 protein - EMEA-000032-PIP02-22

Prevention of meningococcal disease

Day 60 opinion

Action: For adoption

Vaccines

2.1.14. Doxazosin mesilate / finasteride - EMEA-003380-PIP01-22

Treatment and control of benign prostatic hyperplasia

Day 30 opinion

Action: For adoption

Uro-nephrology

2.1.15. Finasteride / tadalafil - EMEA-003323-PIP01-22

Treatment of benign prostate hyperplasia

Day 30 opinion

Action: For adoption

Uro-nephrology

2.1.16. EMEA-003344-PIP02-22

Treatment of amyotrophic lateral sclerosis

Day 30 opinion

Action: For adoption

Neurology

2.2. Opinions on Compliance Check

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

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

Day 60 opinion

Action: For adoption

Dermatology

2.2.2. Alirocumab - EMEA-C-001169-PIP01-11-M05

sanofi-aventis recherche & développement; Treatment of elevated cholesterol

Day 60 opinion

Action: For adoption

Endocrinology-Gynaecology-Fertility-Metabolism

2.2.3. Dasiglucagon - EMEA-C1-002233-PIP01-17-M01

Zealand Pharma a/s; Treatment of hypoglycaemia

Day 60 letter

Action: For adoption

Endocrinology-Gynaecology-Fertility-Metabolism

2.2.4. Bezlotoxumab - EMEA-C-001645-PIP01-14-M04

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

Day 60 opinion

Action: For adoption

Infectious Diseases

2.2.5. Satralizumab - EMEA-C2-001625-PIP01-14-M06

Roche Registration GmbH; Treatment of neuromyelitis optica

Day 60 letter

Action: For adoption

Neurology

2.2.6. Methylphenidate hydrochloride - EMEA-C-003189-PIP01-22-M01

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

Day 7 opinion

Action: For adoption

Psychiatry

The PDCO adopted the opinion by written procedure on 2 February 2023

2.3. Opinions on Modification of an Agreed Paediatric Investigation Plan

2.3.1. Remimazolam - EMEA-001880-PIP02-19-M04

PAION Deutschland GmbH; General anaesthesia / Sedation

Day 60 opinion

Action: For adoption

Anaesthesiology

2.3.2. Sotatercept - Orphan - EMEA-002756-PIP01-19-M02

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

Day 60 opinion

Action: For adoption

Cardiovascular Diseases

Note: Withdrawal request received on 6 February 2023

2.3.3. Delgocitinib - EMEA-002329-PIP02-20-M02

LEO Pharma A/S; Treatment of chronic hand eczema

Day 60 opinion

Action: For adoption

Dermatology

2.3.4. Lebrikizumab - EMEA-002536-PIP01-18-M03

Eli Lilly and Company Limited; Treatment of atopic dermatitis

Day 60 opinion

Action: For adoption

Dermatology

2.3.5. Remibrutinib - EMEA-002582-PIP01-19-M02

Novartis Europharm Limited; Treatment of chronic spontaneous urticaria

Day 60 opinion

Action: For adoption

Dermatology

2.3.6. Denosumab - EMEA-000145-PIP02-12-M05

Amgen Europe B.V.; Treatment of osteoporosis

Day 60 opinion

Action: For adoption

Endocrinology-Gynaecology-Fertility-Metabolism

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

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

Day 60 opinion

Action: For adoption

Endocrinology-Gynaecology-Fertility-Metabolism

2.3.8. Osilodrostat - Orphan - EMEA-000315-PIP02-15-M03

Recordati Rare Diseases SARL; Treatment of adrenal cortical hyperfunction

Day 60 opinion

Action: For adoption

Endocrinology-Gynaecology-Fertility-Metabolism

2.3.9. Obeticholic acid - Orphan - EMEA-001304-PIP02-13-M06

Advanz Pharma Limited; Treatment of biliary atresia / Treatment of primary biliary cirrhosis

Day 60 opinion

Action: For adoption. Oral explanation to be held on Wednesday, 22 February at 14:00

Gastroenterology-Hepatology

2.3.10. Andexanet alfa - EMEA-001902-PIP01-15-M07

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

Day 60 opinion

Action: For adoption

Haematology-Hemostaseology

Note: Withdrawal request received on 3 February 2023

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

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

Day 60 opinion

Action: For adoption

Haematology-Hemostaseology

2.3.12. Filgotinib - EMEA-001619-PIP04-17-M02

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

Day 60 opinion

Action: For adoption

Immunology-Rheumatology-Transplantation

2.3.13. Tofacitinib citrate - EMEA-000576-PIP01-09-M14

Pfizer Europe MA EEIG; Treatment of chronic idiopathic arthritis (including rheumatoid arthritis, psoriatic arthritis, ankylosing spondylitis and juvenile idiopathic arthritis)

Day 60 opinion

Action: For adoption

Immunology-Rheumatology-Transplantation

2.3.14. Lamivudine / dolutegravir - EMEA-001940-PIP01-16-M05

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

Day 60 opinion

Action: For adoption

Infectious Diseases

2.3.15. Oritavancin (diphosphate) - EMEA-001270-PIP01-12-M06

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

Day 60 opinion

Action: For adoption

Infectious Diseases

2.3.16. Tedizolid phosphate - EMEA-001379-PIP01-12-M07

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

Day 60 opinion

Action: For adoption

Infectious Diseases

2.3.17. Eculizumab - Orphan - EMEA-000876-PIP03-14-M06

Alexion Europe SAS; Treatment of neuromyelitis optica spectrum disorders

Day 60 opinion

Action: For adoption

Neurology

2.3.18. Rimegepant - EMEA-002812-PIP02-20-M02

Biohaven Pharmaceutical Ireland DAC; Treatment of migraine headaches

Day 60 opinion

Action: For adoption

Neurology

2.3.19. Satralizumab - Orphan - EMEA-001625-PIP02-21-M01

Roche Registration GmbH; Treatment of myasthenia gravis

Day 60 opinion

Action: For adoption

Neurology

2.3.20. Acalabrutinib - Orphan - EMEA-001796-PIP03-16-M03

Acerta Pharma, BV; Treatment of mature B cell neoplasms

Day 60 opinion

Action: For adoption

Oncology

2.3.21. Carfilzomib - Orphan - EMEA-001806-PIP04-19-M01

Amgen Europe BV; Treatment of acute lymphoblastic leukaemia

Day 60 opinion

Action: For adoption

Oncology

2.3.22. Selpercatinib - EMEA-002544-PIP01-18-M02

Eli Lilly and Company; Treatment of all conditions included in the category of malignant neoplasms (except haematopoietic and lymphoid tissue neoplasms)

Day 60 opinion

Action: For adoption

Oncology

2.3.23. Temozolomide - EMEA-002634-PIP01-19-M02

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

Day 60 opinion

Action: For adoption

Oncology

2.3.24. Botaretigene sparoparvovec - Orphan - EMEA-002827-PIP01-20-M01

Janssen-Cilag International NV Turnhoutseweg 30; Treatment of retinitis pigmentosa

Day 60 opinion

Action: For adoption

Ophthalmology

2.3.25. Patiromer sorbitex calcium - EMEA-001720-PIP01-14-M03

Vifor Fresenius Medical Care Renal Pharma France; Treatment of hyperkalaemia

Day 60 opinion

Action: For adoption

Other

2.3.26. Zilucoplan - Orphan - EMEA-002747-PIP01-20-M01

UCB Pharma SA; Treatment of myasthenia gravis

Day 60 opinion

Action: For adoption

Other / Neurology

2.3.27. Defatted powder of peanuts - EMEA-001734-PIP01-14-M06

Aimmune Therapeutics Inc; Treatment of peanut allergy

Day 60 opinion

Action: For adoption

Pneumology - Allergology

2.3.28. Ravulizumab (ALXN1210) - EMEA-001943-PIP02-20-M02

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

Day 60 opinion

Action: For adoption

Uro-nephrology / Haematology-Hemostaseology

2.3.29. Gadopiclenol - EMEA-001949-PIP01-16-M05

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

Day 30 opinion

Action: For adoption

Diagnostic

2.3.30. Gadopiclenol - EMEA-001949-PIP02-18-M03

Guerbet; Detection and visualization of disorders or lesions with suspected abnormal vascularity in various body regions for diagnostic purposes

Day 30 opinion

Action: For adoption

Diagnostic

2.4. Opinions on Re-examinations

2.4.1. Fordadistrogene movaparovec - Orphan - EMEA-002741-PIP01-20-M01

Pfizer Europe MA EEIG; Treatment of Duchenne muscular dystrophy

Day 30 opinion

Action: For adoption

Neurology

The PDCO adopted the opinion via written procedure on 17 February 2023

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. Dostarlimab - EMEA-C1-002463-PIP01-18-M01

GlaxoSmithKline (Ireland) Limited; Treatment of all conditions included in the category of malignant neoplasms (except haematopoietic and lymphoid malignancies)

Day 30 letter

Action: For information

Oncology

2.7.2. Recombinant human A disintegrin and metalloprotease with thrombospondin type-1 Motifs 13 - EMEA-C1-001160-PIP01-11-M03

Takeda Pharmaceuticals International AG Ireland Branch; Treatment of thrombotic thrombocytopenic purpura

Day 30 letter

Action: For information

Haematology-Hemostaseology

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. EMEA-002612-PIP02-22

Treatment of sickle cell disease

Day 90 discussion

Action: For discussion

Haematology-Hemostaseology

3.1.2. Itolizumab - Orphan - EMEA-003208-PIP02-22

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

Day 90 discussion

Action: For discussion

Immunology-Rheumatology-Transplantation

3.1.3. Albaconazole - EMEA-003279-PIP01-22

Treatment of acute vulvovaginal candidiasis

Day 90 discussion

Action: For discussion

Infectious Diseases

3.1.4. Opelconazole - EMEA-003249-PIP01-22

Treatment of bronchopulmonary aspergillosis / Treatment of invasive aspergillosis with indication limited to bronchopulmonary aspergillosis

Day 90 discussion

Action: For discussion

Infectious Diseases

3.1.5. A 2'-O-(2-methoxyethyl) modified antisense oligonucleotide targeting fused in sarcoma (FUS) pre-mRNA - EMEA-003024-PIP01-21

Treatment of amyotrophic lateral sclerosis (ALS) patients with fused in sarcoma (FUS) mutations (FUS-ALS) ≥ 12 years of age

Day 90 discussion

Action: For discussion

Neurology

3.1.6. Ocrelizumab - EMEA-000310-PIP05-22

Treatment of multiple sclerosis

Day 90 discussion

Action: For discussion

Neurology

3.1.7. Pembrolizumab / favezelimab - EMEA-003104-PIP02-22

Treatment of Hodgkin lymphoma

Day 90 discussion

Action: For discussion

Oncology

3.1.8. Pembrolizumab / vibostolimab - EMEA-003063-PIP02-22

Treatment of Hodgkin lymphoma

Day 90 discussion

Action: For discussion

Oncology

3.1.9. Aticaprant - EMEA-003251-PIP01-22

Treatment of major depressive disorder

Day 90 discussion

Action: For discussion

Psychiatry

3.1.10. Atrasentan - Orphan - EMEA-001666-PIP02-21

Chinook Therapeutics, Inc.; Treatment of IgA nephropathy / Treatment of primary IgA nephropathy

Day 90 discussion

Action: For discussion

Uro-nephrology

3.1.11. Nizaracianine - EMEA-003367-PIP01-22

Enhancement of the ureters during surgery

Day 60 discussion

Action: For discussion

Diagnostic

3.1.12. Venglustat - Orphan - EMEA-001716-PIP07-22

Genzyme Europe B.V.; Treatment of Gaucher disease

Day 60 discussion

Action: For discussion

Endocrinology-Gynaecology-Fertility-Metabolism

3.1.13. Pyridoxal 5'-phosphate monohydrate - Orphan - EMEA-002404-PIP02-22

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

Day 60 discussion

Action: For discussion

Endocrinology-Gynaecology-Fertility-Metabolism / Neurology

3.1.14. Govorestat - Orphan - EMEA-003365-PIP01-22

Applied Therapeutics, Inc; Treatment of galactosemia

Day 60 discussion

Action: For discussion

Endocrinology-Gynaecology-Fertility-Metabolism / Ophthalmology / Neurology

3.1.15. Fazirsiran - Orphan - EMEA-003355-PIP01-22

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

Day 60 discussion

Action: For discussion

Gastroenterology-Hepatology

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

Treatment of ulcerative colitis

Day 60 discussion

Action: For discussion

Gastroenterology-Hepatology

3.1.17. Ruzotolimod - EMEA-003363-PIP01-22

Treatment of chronic hepatitis B

Day 60 discussion

Action: For discussion

Infectious Diseases

3.1.18. Xalnesiran - EMEA-003362-PIP01-22

Treatment of chronic hepatitis B

Day 60 discussion

Action: For discussion

Infectious Diseases

3.1.19. Tozorakimab - EMEA-003360-PIP01-22

Treatment of acute respiratory failure

Day 60 discussion

Action: For discussion

Infectious Diseases / Pneumology - Allergology

3.1.20. Clonidine - EMEA-003198-PIP02-22

Treatment of attention deficit hyperactivity disorder

Day 60 discussion

Action: For discussion

Neurology

3.1.21. EMEA-003271-PIP02-22

Treatment of primary generalised tonic-clonic seizures

Day 60 discussion

Action: For discussion

Neurology

3.1.22. Naxitamab - EMEA-002346-PIP02-22

Treatment of osteosarcoma

Day 60 discussion

Action: For discussion

Oncology

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

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

Day 60 discussion

Action: For discussion

Oncology

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

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

Day 60 discussion

Action: For discussion

Oncology

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

Treatment of malignant neoplasms of the central nervous system

Day 60 discussion

Action: For discussion

Oncology

3.1.26. [Pabinafusp alfa - Orphan - EMEA-003033-PIP02-22](#)

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

Day 60 discussion

Action: For discussion

Other

3.1.27. [Cannabidiol - Orphan - EMEA-003176-PIP02-22](#)

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

Day 60 discussion

Action: For discussion

Psychiatry

3.1.28. Inaxaplin - Orphan - EMEA-003368-PIP01-22

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

Day 60 discussion

Action: For discussion

Uro-nephrology

3.1.29. 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 60 discussion

Action: For discussion

Vaccines

3.1.30. Ezetimibe / atorvastatin - EMEA-003373-PIP01-22

Treatment of homozygous familial hypercholesterolaemia (HoFH) / hypercholesterolaemia

Day 30 discussion

Action: For discussion

Cardiovascular Diseases

3.1.31. Indapamide / ramipril - EMEA-003372-PIP01-22

Treatment of hypertension

Day 30 discussion

Action: For discussion

Cardiovascular Diseases

3.1.32. Upadacitinib - EMEA-001741-PIP08-22

Treatment of hidradenitis suppurativa

Day 30 discussion

Action: For discussion

Dermatology

3.1.33. [Escherichia coli, expressing high affinity phenylalanine transporter, modified phenylalanine ammonia lyase and L-amino acid deaminase - EMEA-003381-PIP01-22](#)

Treatment of hyperphenylalaninemia

Day 30 discussion

Action: For discussion

Endocrinology-Gynaecology-Fertility-Metabolism

3.1.34. [Leuprorelin - EMEA-003354-PIP01-22](#)

Treatment of central (gonadotropin-dependent) precocious puberty

Day 30 discussion

Action: For discussion

Endocrinology-Gynaecology-Fertility-Metabolism

3.1.35. [Cemdisiran - Orphan - EMEA-003237-PIP02-22](#)

Regeneron Ireland DAC; Treatment of paroxysmal nocturnal haemoglobinuria

Day 30 discussion

Action: For discussion

Haematology-Hemostaseology

3.1.36. [Pozelimab - EMEA-003238-PIP02-22](#)

Treatment of paroxysmal nocturnal haemoglobinuria

Day 30 discussion

Action: For discussion

Haematology-Hemostaseology

3.1.37. [Axatilimab - EMEA-003385-PIP01-22](#)

Treatment of chronic graft-versus-host-disease

Day 30 discussion

Action: For discussion

Immunology-Rheumatology-Transplantation

[3.1.38. Nipocalimab - Orphan - EMEA-002559-PIP07-22](#)

Janssen-Cilag International NV; Treatment of Sjögren's syndrome

Day 30 discussion

Action: For discussion

Immunology-Rheumatology-Transplantation

[3.1.39. Inebilizumab - EMEA-001911-PIP02-22](#)

Treatment of generalised myasthenia gravis

Day 30 discussion

Action: For discussion

Neurology

[3.1.40. LY3884963 \(PR006A\) - Orphan - EMEA-003374-PIP01-22](#)

Prevail Therapeutics, a Wholly-Owned Subsidiary of Eli Lilly and Company; Treatment of frontotemporal dementia

Day 30 discussion

Action: For discussion

Neurology

[3.1.41. EMEA-003364-PIP02-22](#)

Treatment of non-small cell lung cancer

Day 30 discussion

Action: For discussion

Oncology

[3.1.42. 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 30 discussion

Action: For discussion

Oncology

3.1.43. N-(4-(4-amino-5-(3-fluoro-4-((4-methylpyrimidin-2-yl)oxy)phenyl)-7-methyl-7H-pyrrolo[2,3-d] pyrimidin-6-yl)phenyl)methacrylamide hydrochloride - Orphan - EMEA-003371-PIP01-22

Relay Therapeutics Inc.; Treatment of cholangiocarcinoma

Day 30 discussion

Action: For discussion

Oncology

3.1.44. EMEA-003370-PIP01-22

Treatment of recurrent metastatic cancers associated with HPV16 infection including head and neck, anal, cervical, vulvar, vaginal and penile squamous cell carcinomas

Day 30 discussion

Action: For discussion

Oncology

3.1.45. Pembrolizumab / vibostolimab - EMEA-003063-PIP03-22

Treatment of melanoma

Day 30 discussion

Action: For discussion

Oncology

3.1.46. A recombinant humanised monoclonal antibody (immunoglobulin gamma-1 with kappa light chains, IgG1κ) directed against integrin alpha V beta 8 produced in Chinese hamster ovary (CHO) cells - EMEA-003376-PIP01-22

Treatment of renal cell carcinoma (RCC) / advanced or metastatic solid tumours / Treatment of head and neck squamous cell carcinoma (HNSCC)

Day 30 discussion

Action: For discussion

Oncology

3.1.47. Taldefgrobep alfa - EMEA-003386-PIP01-22

Treatment of spinal muscular atrophy

Day 30 discussion

Action: For discussion

Other

3.1.48. EMEA-003319-PIP04-22

Treatment of borderline personality disorder (BPD)

Day 30 discussion

Action: For discussion

Psychiatry

3.1.49. *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 A polysaccharide conjugated to tetanus toxoid carrier protein / *Neisseria meningitidis* group C polysaccharide conjugated to tetanus toxoid carrier protein - EMEA-003379-PIP01-22

Prevention of meningococcal disease (serogroups A, B, C, W and Y)

Day 30 discussion

Action: For discussion

Vaccines

3.2. Discussions on Compliance Check

The following compliance checks have been identified for discussion and the members of the PDCO have been invited to comment on issues of possible non-compliance.

3.2.1. Birch bark extract - EMEA-C-001299-PIP03-17-M01

Amryt Pharmaceuticals DAC; Treatment of epidermolysis bullosa

Day 30 discussion

Action: For discussion

Dermatology

3.2.2. Sirolimus - EMEA-C1-003168-PIP01-21

Desitin Arzneimittel GmbH; Treatment of tuberous sclerosis

Day 30 discussion

Action: For discussion

Dermatology

3.2.3. Maralixibat chloride - EMEA-C2-001475-PIP03-17-M03

Mirum Pharmaceuticals; Treatment of progressive familial intrahepatic cholestasis

Day 30 discussion

Action: For discussion

Gastroenterology-Hepatology

3.2.4. [Eltrombopag - EMEA-C-000170-PIP03-13-M04](#)

Novartis Europharm Limited; Treatment of aplastic anaemia

Day 30 discussion

Action: For discussion

Haematology-Hemostaseology

3.2.5. [Givinostat - EMEA-C1-000551-PIP04-21-M01](#)

Italfarmaco S.p.A.; Treatment of Duchenne muscular dystrophy

Day 30 discussion

Action: For discussion

Neurology

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

Sanofi Aventis Groupe; Treatment of multiple sclerosis

Day 30 discussion

Action: For discussion

Neurology

3.2.7. [Entrectinib - EMEA-C-002096-PIP01-16-M03](#)

Roche Registration GmbH; Treatment of all conditions included in the category of malignant neoplasms (except haematopoietic and lymphoid tissue neoplasms)

Day 30 discussion

Action: For discussion

Oncology

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

3.3.1. [Tralokinumab - EMEA-001900-PIP02-17-M07](#)

LEO Pharma A/S; Treatment of atopic dermatitis

Day 30 discussion

Action: For discussion

Dermatology

3.3.2. [Dasiglucagon - EMEA-002233-PIP01-17-M02](#)

Zealand Pharma A/S; Treatment of hypoglycaemia

Day 30 discussion

Action: For discussion

Endocrinology-Gynaecology-Fertility-Metabolism

3.3.3. [Elosulfase alfa - Orphan - EMEA-000973-PIP01-10-M04](#)

BioMarin International Limited; Treatment of mucopolysaccharidosis, type IVA (Morquio A syndrome) in patients of all ages

Day 30 discussion

Action: For discussion

Endocrinology-Gynaecology-Fertility-Metabolism

3.3.4. [Migalastat \(hydrochloride\) - Orphan - EMEA-001194-PIP01-11-M06](#)

Amicus Therapeutics Europe Limited; Treatment of Fabry disease

Day 30 discussion

Action: For discussion

Endocrinology-Gynaecology-Fertility-Metabolism

3.3.5. [Romosozumab - EMEA-001075-PIP04-15-M06](#)

UCB Pharma S.A.; Treatment of osteoporosis

Day 30 discussion

Action: For discussion

Endocrinology-Gynaecology-Fertility-Metabolism

3.3.6. [Tolvaptan - EMEA-001231-PIP02-13-M10](#)

Otsuka Pharmaceutical Netherlands B.V.; Treatment of polycystic kidney disease (PKD)

Day 30 discussion

Action: For discussion

Endocrinology-Gynaecology-Fertility-Metabolism / Uro-nephrology

3.3.7. Guselkumab - EMEA-001523-PIP04-19-M02

Janssen-Cilag International NV; Treatment of ulcerative colitis

Day 30 discussion

Action: For discussion

Gastroenterology-Hepatology

3.3.8. Peptide derivative of glucagon-like-peptide 1 and glucagon with fatty acid side chain - EMEA-002942-PIP02-20-M01

Boehringer Ingelheim International GmbH; Treatment of obesity

Day 30 discussion

Action: For discussion

Gastroenterology-Hepatology

3.3.9. Valoctocogene roxaparvovec - Orphan - EMEA-002427-PIP01-18-M02

BioMarin International Limited; Treatment of haemophilia A

Day 30 discussion

Action: For discussion

Haematology-Hemostaseology

3.3.10. Anifrolumab - EMEA-001435-PIP02-16-M02

AstraZeneca AB; Treatment of systemic lupus erythematosus / lupus nephritis

Day 30 discussion

Action: For discussion

Immunology-Rheumatology-Transplantation

3.3.11. Upadacitinib - EMEA-001741-PIP04-17-M04

AbbVie Ltd; Treatment of atopic dermatitis

Day 30 discussion

Action: For discussion

Immunology-Rheumatology-Transplantation / Dermatology

3.3.12. Gepotidacin - EMEA-002443-PIP01-18-M02

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

Day 30 discussion

Action: For discussion

Infectious Diseases

3.3.13. [Maribavir - Orphan - EMEA-000353-PIP02-16-M03](#)

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

Day 30 discussion

Action: For discussion

Infectious Diseases

3.3.14. [Efgartigimod alfa - Orphan - EMEA-002597-PIP05-21-M01](#)

argenx BV; Treatment of myasthenia gravis

Day 30 discussion

Action: For discussion

Neurology

3.3.15. [Lasmiditan - EMEA-002166-PIP01-17-M07](#)

Eli Lilly and Company Limited; Treatment of migraine with and without aura

Day 30 discussion

Action: For discussion

Neurology

3.3.16. [Vatiquinone - Orphan - EMEA-001238-PIP03-21-M01](#)

PTC Therapeutics International; Treatment of Friedreich's ataxia

Day 30 discussion

Action: For discussion

Neurology

3.3.17. [Binimetinib - EMEA-001454-PIP03-15-M03](#)

Pierre Fabre Médicament; Treatment of melanoma

Day 30 discussion

Action: For discussion

Oncology

3.3.18. Encorafenib - EMEA-001588-PIP01-13-M03

Pierre Fabre Médicament; Treatment of melanoma

Day 30 discussion

Action: For discussion

Oncology

3.3.19. Isatuximab - EMEA-002205-PIP01-17-M04

Sanofi-Aventis Recherche & Développement; Treatment of malignant neoplasms of the haematopoietic and lymphoid tissue

Day 30 discussion

Action: For discussion

Oncology

3.3.20. Repotrectinib - EMEA-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

3.3.21. Autologous CD34+ hematopoietic stem and progenitor cells (HSPCs) genetically modified with the lentiviral vector IDUA LVV, encoding for the human α -L-iduronidase (IDUA) gene - Orphan - EMEA-003001-PIP01-21-M01

Orchard Therapeutics (Netherlands) B.V.; Treatment of mucopolysaccharidosis type I, Hurler syndrome

Day 30 discussion

Action: For discussion

Other

3.3.22. Iptacopan - Orphan - EMEA-002705-PIP01-19-M01

Novartis Europharm Limited; Treatment of C3 glomerulopathy

Day 30 discussion

Action: For discussion

Other

3.3.23. Meloxicam / bupivacaine - EMEA-002246-PIP01-17-M03

Heron Therapeutics B.V.; Treatment of acute postoperative pain

Day 30 discussion

Action: For discussion

Pain

3.3.24. Benralizumab - EMEA-001214-PIP09-21-M01

AstraZeneca AB; Treatment of eosinophilic granulomatosis with polyangiitis (EGPA)

Day 30 discussion

Action: For discussion

Pneumology - Allergology

3.3.25. Sodium chloride / idrevloride - Orphan - EMEA-002935-PIP01-20-M03

Parion Sciences, Inc.; Treatment of primary ciliary dyskinesia (PCD)

Day 30 discussion

Action: For discussion

Pneumology - Allergology

3.3.26. Finerenone - EMEA-001623-PIP01-14-M06

Bayer AG; Treatment of chronic kidney disease

Day 30 discussion

Action: For discussion

Uro-nephrology

3.3.27. Sparsentan - Orphan - EMEA-001984-PIP02-20-M01

Vifor (International) AG; Treatment of focal segmental glomerular sclerosis (FSGS)

Day 30 discussion

Action: For discussion

Uro-nephrology

3.3.28. Cholera vaccine, recombinant, live, oral (strain CVD 103-HgR) - EMEA-001490-PIP01-13-M04

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

Day 30 discussion

Action: For discussion

Vaccines

4. Nominations

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

4.1. List of submissions of applications with start of procedure 27 February 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. N-(3-{6-Amino-5-[2-(N-methylprop-2-enamido)ethoxy]pyrimidin-4-yl}-5-fluoro-2-methylphenyl)-4-cyclopropyl-2-fluorobenzamide - EMEA-002582-PIP01-19

Novartis Pharma AG; Treatment of chronic spontaneous urticaria

Action: For adoption

7.1.2. Human papillomavirus type 6 L1 protein / human papillomavirus type 11 L1 protein / human papillomavirus type 16 L1 protein / human papillomavirus type 18 L1 protein / human papillomavirus type 31 L1 protein / human papillomavirus type 33 L1 protein / human papillomavirus type 45 L1 protein / human papillomavirus type 52 L1 protein / human papillomavirus type 58 L1 protein - EMEA-000654-PIP01-09-M02

Merck Sharp & Dohme (Europe), Inc.; Prevention of infection by human papillomavirus

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) – Uppsala, 7-8 June 2023

No item

9.2. Coordination with EMA Scientific Committees or CMDh-v

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

Action: For information

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

9.3.1. Non-clinical Working Party: D30 Products identified

PDCO member: Karen van Malderen

Action: For information

9.3.2. 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 – 3 March 2023

Meeting summary – PCWP/HCPWP annual meeting with all eligible organisations – 15 November 2022

Action: For information

9.4. Cooperation within the EU regulatory network

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

No item

9.5. Cooperation with International Regulators

9.5.1. Paediatric Cluster Teleconference

Action: For information

9.6. Contacts of the PDCO with external parties and interaction with the Interested Parties to the Committee

No item

9.7. PDCO work plan

No Item

9.8. Planning and reporting

9.8.1. EMA Business Pipeline activity and Horizon scanning

No item

10. Any other business

10.1. Feedback from MRCT conference on 'Aligning Stakeholders to Facilitate Pediatric Access to Medicines: From Product Development through HTA Review'

PDCO members: Sylvie Benchetrit, Siri Wang and Sabine Scherer

Action: For information

10.2. Oncology European Specialised Expert Community (ESEC) webinar on acute myeloid leukaemia (AML)

Action: For information

10.3. Overview of ITF activities in 2022: Trends and topics in focus

Action: For information

10.4. PDCO requirement for modifying allergen PIPs

PDCO member: Yuansheng Sun

Action: For information

10.5. Real World Evidence update, including DARWIN EU®

Action: For information

10.6. Upcoming Innovation Task Force (ITF) meetings

Action: For information

10.7. Update of activities Oncology European Specialised Expert Community (ESEC)

Action: For information

11. Breakout sessions

11.1. Paediatric oncology

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

11.2. Neonatology

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

11.3. HIV

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/