



EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

16 February 2024
EMA/PDCO/35476/2024
Human Medicines Division

Paediatric Committee (PDCO)

Draft Agenda for the meeting on 20-23 February 2024

Chair: Brian Aylward – Vice-Chair: Sylvie Benchetrit

20 February 2024, 14:00 - 19:00, Virtual meeting

21 February 2024, 08:30 - 19:00, Virtual meeting

22 February 2024, 08:30 - 19:00, Virtual meeting

23 February 2024, 08:30 - 13:00, Virtual meeting

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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 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 20-23 February 2024. See February 2024 PDCO minutes (to be published post March 2024 PDCO meeting).

1.2. Adoption of agenda

PDCO agenda for 20-23 February 2024.

1.3. Adoption of the minutes

PDCO minutes for 16-19 January 2024.

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. Navepegritide - Orphan - EMEA-002689-PIP02-23

Ascendis Pharma Growth Disorders A/S; Treatment of achondroplasia

Day 120 opinion

Action: For adoption

Endocrinology-Gynaecology-Fertility-Metabolism

2.1.2. Orforglipron - EMEA-003299-PIP01-22

Treatment of type 2 diabetes mellitus

Day 120 opinion

Action: For adoption

Endocrinology-Gynaecology-Fertility-Metabolism

2.1.3. Belimumab - EMEA-000520-PIP03-23

Treatment of systemic sclerosis

Day 120 opinion

Action: For adoption

Immunology-Rheumatology-Transplantation

2.1.4. Iodine (¹³¹I) apamistamab - Orphan - EMEA-003395-PIP02-23

Immedica Pharma AB; Treatment in allogenic stem cell transplantation

Day 120 opinion

Action: For adoption

Immunology-Rheumatology-Transplantation / Oncology

2.1.5. Trimodulin (human IgM, IgA, IgG solution) - EMEA-002883-PIP03-23

Treatment of lower respiratory tract and lung infections

Day 120 opinion

Action: For adoption

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

2.1.6. EMEA-003477-PIP01-23

Treatment of appetite and general nutrition disorders

Day 120 opinion

Action: For adoption

Nutrition

Note: Withdrawal request received on 6 February 2024

2.1.7. Disitamab vedotin - EMEA-003443-PIP02-23

Treatment of HER2 expressing tumours / Treatment of solid tumours

Day 120 opinion

Action: For adoption

Oncology

2.1.8. Alpelisib - Orphan - EMEA-002016-PIP05-23

Novartis Europharm Limited; Treatment of lymphatic malformations associated with a

PIK3CA mutation

Day 120 opinion

Action: For adoption

Other

2.1.9. Garetosmab - Orphan - EMEA-002736-PIP02-23

Regeneron Ireland DAC; Treatment of fibrodysplasia ossificans progressiva

Day 120 opinion

Action: For adoption

Other

2.1.10. Losmapimod - Orphan - EMEA-003448-PIP01-23

Fulcrum Therapeutics, Inc.; Treatment of facioscapulohumeral muscular dystrophy

Day 120 opinion

Action: For adoption

Other

2.1.11. EMEA-003394-PIP01-23

Treatment of dystrophinopathies

Day 120 opinion

Action: For adoption

Other / Neurology

2.1.12. Cannabidiol - Orphan - EMEA-003176-PIP02-22

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

Day 120 opinion

Action: For adoption

Psychiatry

2.1.13. [MRNA encoding CMV gB / mRNA encoding the gH protein in the CMV glycoprotein complex pentamer / mRNA encoding the UL131A protein in the CMV glycoprotein complex pentamer / mRNA encoding the UL130 protein in the CMV glycoprotein complex pentamer / mRNA encoding the gL protein in the CMV glycoprotein complex pentamer / mRNA encoding the UL128 protein in the CMV glycoprotein complex pentamer - EMEA-003405-PIP01-23](#)

Prevention of cytomegalovirus infection

Day 120 opinion

Action: For adoption

Vaccines

2.1.14. [Ramipril / nebivolol - EMEA-003530-PIP01-23](#)

Treatment of hypertension / Treatment of heart failure / Treatment of coronary artery disease / Treatment of hypertension with coexisting heart failure / Treatment of hypertension with coexisting coronary artery disease

Day 60 opinion

Action: For adoption

Cardiovascular Diseases

2.1.15. [Human IgG1 monoclonal antibody targeting amyloid transthyretin - EMEA-003548-PIP01-23](#)

Treatment of transthyretin amyloidosis (ATTR)

Day 60 opinion

Action: For adoption

Cardiovascular Diseases / Neurology

2.1.16. [Urea / propylene glycol - EMEA-003542-PIP01-23](#)

Treatment of dry skin

Day 60 opinion

Action: For adoption

Dermatology

2.1.17. [Dapagliflozin / sitagliptin - EMEA-003534-PIP01-23](#)

Treatment of type II diabetes mellitus

Day 60 opinion

Action: For adoption

Endocrinology-Gynaecology-Fertility-Metabolism

[2.1.18. Efgartigimod alfa - EMEA-002597-PIP10-23](#)

Treatment of thyroid eye disease

Day 60 opinion

Action: For adoption

Endocrinology-Gynaecology-Fertility-Metabolism

[2.1.19. Human HSD17B1 enzyme inhibitor - EMEA-003537-PIP01-23](#)

Treatment of endometriosis

Day 60 opinion

Action: For adoption

Endocrinology-Gynaecology-Fertility-Metabolism

[2.1.20. 2-\[4-Methoxy-3-\(2-m-tolyl-ethoxy\)-benzoylamino\]-indan-2-carboxylic acid \(fipaxalparant\) - Orphan - EMEA-003539-PIP01-23](#)

Horizon Therapeutics Ireland DAC; Treatment of progressive fibrosing interstitial lung disease

Day 60 opinion

Action: For adoption

Immunology-Rheumatology-Transplantation

[2.1.21. Empasiprubart - EMEA-003528-PIP01-23](#)

Treatment of multifocal motor neuropathy

Day 60 opinion

Action: For adoption

Neurology

[2.1.22. Suvecaltamide - EMEA-003248-PIP02-23](#)

Treatment of moderate to severe tremor in patients with Parkinson's disease

Day 60 opinion

Action: For adoption

Neurology

2.1.23. Ursodoxicoltaurine / sodium phenylbutyrate - Orphan - EMEA-002876-PIP02-23

Amylyx Pharmaceuticals EMEA B.V.; Treatment of progressive supranuclear palsy

Day 60 opinion

Action: For adoption

Neurology

2.1.24. Autologous T-cells expressing a chimeric antigenic receptor against G protein coupled receptor family C group 5 member D (GPC5D) - EMEA-003543-PIP01-23

Treatment of multiple myeloma

Day 60 opinion

Action: For adoption

Oncology

2.1.25. Zongertinib - EMEA-003546-PIP01-23

Treatment of non-small cell lung cancer

Day 60 opinion

Action: For adoption

Oncology

2.1.26. Palonosetron / netupitant - EMEA-001198-PIP04-23

Prevention of chemotherapy-induced nausea and vomiting

Day 60 opinion

Action: For adoption

Other

2.1.27. Vixarelimab - EMEA-003540-PIP01-23

Treatment of idiopathic pulmonary fibrosis

Day 60 opinion

Action: For adoption

Pneumology – Allergology

2.1.28. Maplirpcept - EMEA-003551-PIP01-23

Treatment of multiple myeloma

Day 30 opinion

Action: For adoption

Oncology

2.1.29. [Influenza virus type B, Victoria lineage / influenza virus type A, H3N2 / influenza virus type A, H1N1 - EMEA-003589-PIP01-24](#)

Prevention of influenza infection

Day 1 opinion

Action: For information

Vaccines

The PDCO adopted the opinion by written procedure on 8 February 2024

2.1.30. [Povorcitinib - EMEA-003313-PIP03-23](#)

Treatment of prurigo nodularis

Day 30 opinion

Action: For adoption

Dermatology

2.2. **Opinions on Compliance Check**

2.2.1. [Letemovir - EMEA-C-001631-PIP01-14-M05](#)

Merck Sharp & Dohme B.V.; Prevention of cytomegalovirus infection

Day 60 opinion

Action: For adoption

Infectious Diseases

2.2.2. [Tedizolid phosphate - EMEA-C2-001379-PIP01-12-M08](#)

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

Day 60 letter

Action: For adoption

Infectious Diseases

2.2.3. [Hydrocortisone - EMEA-C-002305-PIP01-17-M01](#)

Laboratoire AGUETTANT; Prevention of bronchopulmonary dysplasia

Day 60 opinion

Action: For adoption

Neonatology - Paediatric Intensive Care

2.2.4. Blinatumomab - EMEA-C-000574-PIP02-12-M04

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

Day 60 opinion

Action: For adoption

Oncology

2.2.5. Evinacumab - EMEA-C-002298-PIP01-17-M05

Ultragenyx Germany GmbH; Treatment of elevated cholesterol

Day 30 opinion

Action: For adoption

Endocrinology-Gynaecology-Fertility-Metabolism / Cardiovascular Diseases

2.2.6. Macitentan - EMEA-C-001032-PIP01-10-M07

Janssen-Cilag International NV; Treatment of pulmonary arterial hypertension

Day 30 opinion

Action: For adoption

Cardiovascular Diseases

2.2.7. Avacopan - EMEA-C5-002023-PIP01-16-M07

Amgen Europe B.V.; Treatment of antineutrophil cytoplasmic antibody (ANCA)-associated vasculitis

Day 60 letter

Action: For adoption

Immunology-Rheumatology-Transplantation

2.2.8. Human fibrinogen concentrate (BT524) - EMEA-C-001931-PIP01-16-M03

Biotest AG; Treatment of congenital fibrinogen deficiency

Day 30 opinion

Action: For adoption

Haematology-Hemostaseology

2.2.9. Risdiplam - EMEA-C-002070-PIP01-16-M07

Roche Pharma AG; Treatment of spinal muscular atrophy

Day 30 opinion

Action: For adoption

Neurology

2.3. Opinions on Modification of an Agreed Paediatric Investigation Plan

2.3.1. Chlorprocaine (hydrochloride) - EMEA-000639-PIP03-16-M03

Sintetica GmbH; Peripheral nerve block (local anaesthesia by perineural injection)

Day 60 opinion

Action: For adoption

2.3.2. Aficamten - EMEA-002958-PIP01-21-M01

Cytokinetics, Inc.; Treatment of hypertrophic cardiomyopathy

Day 60 opinion

Action: For adoption

Cardiovascular Diseases

2.3.3. Milvexian - EMEA-003220-PIP01-22-M01

Janssen-Cilag International N.V.; Prevention of thromboembolism in patients with cardiovascular diseases

Day 60 opinion

Action: For adoption

Cardiovascular Diseases

2.3.4. Ruxolitinib phosphate - EMEA-002618-PIP02-20-M01

Incyte Biosciences Distribution B.V.; Treatment of vitiligo

Day 60 opinion

Action: For adoption

Dermatology

2.3.5. [Glucagon analogue linked to a human immunoglobulin Fc fragment - Orphan - EMEA-003170-PIP01-21-M02](#)

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

Day 60 opinion

Action: For adoption

Endocrinology-Gynaecology-Fertility-Metabolism

2.3.6. [Fidanacogene elaparvovec - Orphan - EMEA-002362-PIP02-19-M03](#)

Pfizer Europe MA EEIG; Treatment of congenital factor IX deficiency (haemophilia B)

Day 60 opinion

Action: For adoption

Haematology-Hemostaseology

2.3.7. [Filgotinib - EMEA-001619-PIP04-17-M03](#)

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

2.3.8. [BNT162b2 / tozinameran / famtozinameran / riltozinameran / raxtozinameran - EMEA-002861-PIP02-20-M07](#)

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

Day 60 opinion

Action: For adoption

Infectious Diseases

2.3.9. [1-\[\[\[4-\(4-fluoro-2-methyl-1H-indol-5-yloxy\)-6-methoxyquinolin-7-yl\]oxy\]methyl\]cyclopropanamine-dihydrochloride - Orphan - EMEA-002486-PIP04-21-M01](#)

Advenchen Laboratories, LLC; Treatment of Ewing sarcomas / Treatment of soft tissue sarcomas

Day 60 opinion

Action: For adoption

Oncology

2.3.10. Cedazuridine / decitabine - EMEA-003071-PIP01-21-M01

Otsuka Pharmaceutical Netherlands B.V.; Treatment of acute myeloid leukaemia

Day 60 opinion

Action: For adoption

Oncology / Haematology-Hemostaseology

2.3.11. Vamikibart - EMEA-003215-PIP01-22-M01

Roche Registration GmbH; Treatment of macular oedema

Day 60 opinion

Action: For adoption

Ophthalmology

2.3.12. Afamelanotide - Orphan - EMEA-000737-PIP02-11-M03

Clinuvel Europe Limited; Treatment of erythropoietic protoporphyria

Day 60 opinion

Action: For adoption

Other

2.3.13. In vitro expanded autologous human articular chondrocytes - EMEA-001823-PIP01-15-M03

TETEC Tissue Engineering Technologies AG; Treatment of cartilage disorders

Day 60 opinion

Action: For adoption

Other

2.3.14. 12 Grass pollen extract and cultivated rye pollen extract - EMEA-000813-PIP01-09-M01

Allergy Therapeutics (UK) Ltd.; Treatment of allergic rhinitis/rhino-conjunctivitis

Day 60 opinion

Action: For adoption

Pneumology - Allergology

2.3.15. Seltorexant - EMEA-002746-PIP01-20-M03

Janssen-Cilag International NV; Treatment of major depressive disorder

Day 60 opinion

Action: For adoption. Oral explanation to be held on Wednesday 21 February 2024 at 14:00

Psychiatry

2.3.16. [L-Carnitine / glucose / calcium chloride dihydrate / magnesium chloride hexahydrate / sodium lactate / sodium chloride - EMEA-003049-PIP01-21-M01](#)

Iperboreal Pharma Srl; Treatment of patients in need of peritoneal dialysis

Day 60 opinion

Action: For adoption

Uro-nephrology

2.3.17. [Mirabegron - EMEA-000597-PIP03-15-M06](#)

Astellas Pharma Europe B.V.; Treatment of neurogenic detrusor overactivity

Day 60 opinion

Action: For adoption

Uro-nephrology

2.3.18. [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-M03](#)

GlaxoSmithKline Biologicals SA; Prevention of meningococcal meningitis

Day 60 opinion

Action: For adoption

Vaccines

2.3.19. [Baricitinib - EMEA-001220-PIP01-11-M10](#)

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

Day 30 opinion

Action: For adoption

2.4. Opinions on Re-examinations

2.4.1. Frexalimab - EMEA-002945-PIP03-23

Sanofi Winthrop Industrie; Treatment of type 1 diabetes mellitus

Day 30 opinion

Action: For information

Endocrinology-Gynaecology-Fertility-Metabolism

The PDCO adopted the opinion by written procedure on 14 February 2024

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. Baricitinib - EMEA-C3-001220-PIP01-11-M08

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

Day 30 letter

Action: For information

Immunology-Rheumatology-Transplantation

2.7.2. Vosorotide - EMEA-C6-002033-PIP01-16-M03

BioMarin International Limited; Treatment of achondroplasia

Day 15 letter

Action: For information

Other

2.7.3. Obeticholic acid - EMEA-C2-001304-PIP02-13-M07

Advanz Pharma Limited; Treatment of biliary atresia

Day 30 letter

Action: For information

Gastroenterology-Hepatology

2.7.4. Ferric citrate coordination complex (FCCC) - EMEA-C1-001213-PIP03-23

Averoa SAS; Treatment of anaemias due to chronic kidney disorders

Day 30 letter

Action: For information

Uro-nephrology

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

Treatment of hypercholesterolaemia

Day 90 discussion

Action: For discussion

Cardiovascular Diseases

3.1.2. Lutikizumab - EMEA-003481-PIP01-23

Treatment of hidradenitis suppurativa

Day 90 discussion

Action: For discussion

Dermatology

3.1.3. Spesolimab - EMEA-002475-PIP04-23

Treatment of hidradenitis suppurativa

Day 90 discussion

Action: For discussion

Dermatology

3.1.4. [GIPR antagonist/GLP-1R agonist - EMEA-003439-PIP02-23](#)

Treatment of obesity

Day 90 discussion

Action: For discussion

Endocrinology-Gynaecology-Fertility-Metabolism

3.1.5. [Plecanatide - EMEA-003441-PIP01-23](#)

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

Day 90 discussion

Action: For discussion

Gastroenterology-Hepatology

3.1.6. [Etavopivat - Orphan - EMEA-002924-PIP02-23](#)

Novo Nordisk A/S; Treatment of sickle cell disease

Day 90 discussion

Action: For discussion

Haematology-Hemostaseology

3.1.7. [Tozorakimab - EMEA-003360-PIP01-22](#)

Treatment of acute respiratory failure

Day 90 discussion

Action: For discussion

Infectious Diseases / Pneumology - Allergology

3.1.8. [Relatlimab / nivolumab - EMEA-002727-PIP03-23](#)

Treatment of melanoma

Day 90 discussion

Action: For discussion

Oncology

3.1.9. Laruparetigene zovaparvovec - Orphan - EMEA-003457-PIP01-23

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

Day 90 discussion

Action: For discussion

Ophthalmology

3.1.10. Hemopexin, human - Orphan - EMEA-003333-PIP01-22

CSL Behring GmbH; Treatment of sickle cell disease

Day 90 discussion

Action: For discussion

Other

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

Intellia Therapeutics, Inc.; Treatment of hereditary angioedema (HAE)

Day 90 discussion

Action: For discussion

Other

3.1.12. Mometasone - EMEA-003454-PIP01-23

Treatment of chronic rhinosinusitis (CRS)

Day 90 discussion

Action: For discussion

Oto-rhino-laryngology

3.1.13. Tanimilast - EMEA-003393-PIP01-23

Treatment of asthma

Day 90 discussion

Action: For discussion

Pneumology - Allergology

3.1.14. Live attenuated respiratory syncytial virus (RSV) - EMEA-003277-PIP02-23

Prevention of respiratory syncytial virus (RSV) disease

Day 90 discussion

Action: For discussion

Vaccines

3.1.15. [EMEA-003531-PIP01-23](#)

Treatment of hyperphenylalaninemia

Day 60 discussion

Action: For discussion

Endocrinology-Gynaecology-Fertility-Metabolism

3.1.16. [Efinopegdutide - EMEA-003549-PIP01-23](#)

Treatment of non-alcoholic steatohepatitis

Day 60 discussion

Action: For discussion

Gastroenterology-Hepatology

3.1.17. [Ensitrelvir - EMEA-003192-PIP03-23](#)

Post exposure prophylaxis of coronavirus disease 2019 (COVID-19)

Day 60 discussion

Action: For discussion

Infectious Diseases

3.1.18. [Glenzocimab - EMEA-003536-PIP01-23](#)

Treatment of acute ischemic stroke (AIS)

Day 60 discussion

Action: For discussion

Neurology

3.1.19. [Unasnemab - EMEA-003529-PIP01-23](#)

Treatment of spinal cord injury

Day 60 discussion

Action: For discussion

Neurology

3.1.20. Budigalimab - EMEA-003532-PIP01-23

Treatment of solid tumours

Day 60 discussion

Action: For discussion

Oncology

3.1.21. Livmoniplimab - EMEA-003533-PIP01-23

Treatment of solid tumours

Day 60 discussion

Action: For discussion

Oncology

3.1.22. Fragment antibody targeting human TfR1 conjugated to phosphorodiamidate morpholino oligomer - EMEA-003538-PIP01-23

Treatment of Duchenne muscular dystrophy

Day 60 discussion

Action: For discussion

Other

3.1.23. Gastric inhibitory peptide (GIP), glucagon like peptide (GLP) and Gcg tri-agonist - EMEA-003258-PIP02-23

Treatment of obesity

Day 60 discussion

Action: For discussion

Other

3.1.24. Atorvastatin / fenofibrate - EMEA-003563-PIP01-23

Treatment of mixed (or combined) dyslipidaemia

Day 30 discussion

Action: For discussion

Cardiovascular Diseases

3.1.25. Fenofibrate / ezetimibe / rosuvastatin - EMEA-003562-PIP01-23

Treatment of mixed (or combined) dyslipidaemia

Day 30 discussion

Action: For discussion

Cardiovascular Diseases

3.1.26. Povorcitinib - EMEA-003313-PIP02-23

Treatment of vitiligo

Day 30 discussion

Action: For discussion

Dermatology

3.1.27. Sonelokimab - EMEA-002568-PIP02-23

Treatment of hidradenitis suppurativa

Day 30 discussion

Action: For discussion

Dermatology

3.1.28. Linaprazan - EMEA-003558-PIP01-23

Treatment of gastro-oesophageal reflux disease

Day 30 discussion

Action: For discussion

Gastroenterology-Hepatology

3.1.29. Volixibat - EMEA-003567-PIP01-23

Treatment of primary biliary cholangitis

Day 30 discussion

Action: For discussion

Gastroenterology-Hepatology

3.1.30. Humanised IgG4 monoclonal antibody against FIXa and FX - EMEA-003550-PIP01-23

Treatment of coagulation factor deficiencies

Day 30 discussion

Action: For discussion

Haematology-Hemostaseology

3.1.31. Sargramostim - EMEA-003568-PIP01-23

Treatment for patients acutely exposed to myelosuppressive doses of radiation (hematopoietic syndrome of acute radiation syndrome [H-ARS])

Day 30 discussion

Action: For discussion

Immunology-Rheumatology-Transplantation

3.1.32. Tulisokibart - EMEA-003556-PIP01-23

Treatment of ulcerative colitis

Day 30 discussion

Action: For discussion

Immunology-Rheumatology-Transplantation

3.1.33. Human rabies immune globulin - EMEA-003552-PIP01-23

Prevention of rabies viral infection

Day 30 discussion

Action: For discussion

Infectious Diseases

3.1.34. Olorofim - Orphan - EMEA-003564-PIP01-23

Shionogi BV; Treatment of fungal infectious disorders

Day 30 discussion

Action: For discussion

Infectious Diseases

3.1.35. Acetylcysteine - EMEA-003554-PIP01-23

Treatment of hereditary cystatin C amyloid angiopathy

Day 30 discussion

Action: For discussion

Neurology

3.1.36. *Clostridium botulinum* neurotoxin type A (150 kD), free from complexing proteins - EMEA-001039-PIP04-23

Treatment of essential tremor

Day 30 discussion

Action: For discussion

Neurology

3.1.37. [N-{\(2S,3R\)-4,4-difluoro-1-\(2-hydroxy-2-methylpropanoyl\)-2-\[\(2,3',5'-trifluoro\[1,1'-biphenyl\]-3-yl\)methyl\]pyrrolidin-3-yl}ethanesulfonamide - Orphan - EMEA-003553-PIP01-23](#)

Takeda Pharma A/S; Treatment of idiopathic hypersomnia / Treatment of narcolepsy

Day 30 discussion

Action: For discussion

Neurology

3.1.38. [Amivantamab - EMEA-002573-PIP02-23](#)

Treatment of colorectal cancer

Day 30 discussion

Action: For discussion

Oncology

3.1.39. [Camreluzimab - EMEA-003566-PIP01-23](#)

Treatment of hepatocellular carcinoma

Day 30 discussion

Action: For discussion

Oncology

3.1.40. [Erdafitinib - EMEA-002042-PIP03-23](#)

Treatment of urothelial carcinoma

Day 30 discussion

Action: For discussion

Oncology

3.1.41. [Idroxiolic acid, sodium - Orphan - EMEA-003565-PIP01-23](#)

Laminar Pharmaceuticals SA; Treatment of malignant glioma in children, including paediatric-type diffuse high-grade glioma

Day 30 discussion

Action: For discussion

Oncology

3.1.42. [Modified messenger ribonucleic acid encoding individual patient-specific tumour neoantigens - EMEA-003434-PIP02-23](#)

Treatment of renal neoplasms / Treatment of urothelial carcinomas / Treatment of cutaneous squamous cell carcinoma

Day 30 discussion

Action: For discussion

Oncology

3.1.43. [Mosunetuzumab - Orphan - EMEA-002524-PIP03-23](#)

Roche Registration GmbH; Treatment of mature B-cell neoplasms

Day 30 discussion

Action: For discussion

Oncology

3.1.44. [EMEA-003485-PIP01-23](#)

Treatment of non-small cell lung cancer

Day 30 discussion

Action: For discussion

Oncology

3.1.45. [Petosemtamab - EMEA-003557-PIP01-23](#)

Treatment of head and neck epithelial neoplasms

Day 30 discussion

Action: For discussion

Oncology

3.1.46. [Raludotatug deruxtecan - EMEA-003569-PIP01-23](#)

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

Day 30 discussion

Action: For discussion

Oncology

3.1.47. Riletamotide / tapderimotide / alrefimotide - EMEA-003555-PIP01-23

Treatment of malignant mesothelioma

Day 30 discussion

Action: For discussion

Oncology

3.1.48. Carbachol / brimonidine - EMEA-003561-PIP01-23

Treatment of presbyopia

Day 30 discussion

Action: For discussion

Ophthalmology

3.1.49. Rilonecept - Orphan - EMEA-003571-PIP01-23

Kiniksa Pharmaceuticals (UK), Ltd.; Treatment of idiopathic pericarditis

Day 30 discussion

Action: For discussion

Other / Cardiovascular Diseases

3.1.50. Atogepant - EMEA-002530-PIP02-23

Treatment of migraine headaches

Day 30 discussion

Action: For discussion

Pain

3.1.51. Human alpha-1-proteinase inhibitor immunoglobulin G fusion protein, recombinant - EMEA-003570-PIP01-23

Treatment of emphysema secondary to congenital deficiency of alpha-1 antitrypsin

Day 30 discussion

Action: For discussion

Pneumology - Allergology

3.1.52. Dapagliflozin propanediol / baxdrostat - EMEA-003559-PIP01-23

Treatment of chronic kidney disease

Day 30 discussion

Action: For discussion

Uro-nephrology

3.1.53. EMEA-003560-PIP01-23

Prevention of influenza disease and coronavirus disease 2019 (COVID-19)

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. Apraglutide - EMEA-C1-003016-PIP01-21

VectivBio AG; Treatment of short bowel syndrome

Day 30 discussion

Action: For discussion

Gastroenterology-Hepatology

3.2.2. Mozafancogene autotemcel - EMEA-C1-002578-PIP01-19-M01

Rocket Pharmaceuticals, Inc.; Treatment of Fanconi anaemia subtype A

Day 30 discussion

Action: For discussion

Haematology-Hemostaseology

3.2.3. Tasimelteon - EMEA-C1-001531-PIP01-13-M04

Vanda Pharmaceuticals Netherlands B.V.; Treatment of non-24-hour sleep-wake disorder in the totally blind

Day 30 discussion

Action: For discussion

Neurology

3.2.4. Brexucabtagene autoleucel - EMEA-C-001862-PIP01-15-M03

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

Day 30 discussion

Action: For discussion

Oncology

Note: Withdrawal request received on 29 January 2024

3.2.5. [Bilastine - EMEA-C-000347-PIP02-16-M05](#)

Faes Farma, S.A.; Treatment of allergic conjunctivitis

Day 30 discussion

Action: For discussion

Ophthalmology / Pneumology - Allergology

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

3.3.1. [Apixaban - EMEA-000183-PIP02-12-M05](#)

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

Day 30 discussion

Action: For discussion

Cardiovascular Diseases

3.3.2. [Levonorgestrel - EMEA-002474-PIP02-18-M02](#)

Chemo Research, S.L.; Contraception

Day 30 discussion

Action: For discussion

Endocrinology-Gynaecology-Fertility-Metabolism

3.3.3. [Venglustat - Orphan - EMEA-001716-PIP07-22-M01](#)

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

Day 30 discussion

Action: For discussion

Endocrinology-Gynaecology-Fertility-Metabolism

3.3.4. [Avatrombopag maleate - EMEA-001136-PIP01-11-M03](#)

Swedish Orphan Biovitrum AB; Treatment of idiopathic thrombocytopenic purpura /
Treatment of thrombocytopenic purpura secondary to liver disease

Day 30 discussion

Action: For discussion

Haematology-Hemostaseology

3.3.5. Giroctocogene fitelparvovec - Orphan - EMEA-002724-PIP01-19-M03

Pfizer Europe MA EEIG; Treatment of haemophilia A

Day 30 discussion

Action: For discussion

Haematology-Hemostaseology

3.3.6. Pegcetacoplan - Orphan - EMEA-002600-PIP01-19-M02

Swedish Orphan Biovitrum AB (publ); Treatment of paroxysmal nocturnal haemoglobinuria

Day 30 discussion

Action: For discussion

Haematology-Hemostaseology

3.3.7. Onasemnogene abeparvovec - Orphan - EMEA-002168-PIP01-17-M06

Novartis Europharm Limited; Treatment of spinal muscular atrophy

Day 30 discussion

Action: For discussion

Neurology

3.3.8. Brexucabtagene autoleucel - Orphan - EMEA-001862-PIP03-20-M02

Kite Pharma EU B.V.; Treatment of mature B-cell neoplasms

Day 30 discussion

Action: For discussion

Oncology

3.3.9. Loncastuximab tesirine - EMEA-002665-PIP02-20-M01

Swedish Orphan Biovitrum AB (publ); Treatment of mature B-cell neoplasms

Day 30 discussion

Action: For discussion

Oncology

3.3.10. Nivolumab - EMEA-001407-PIP02-15-M07

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

Day 30 discussion

Action: For discussion

Oncology

3.3.11. Olaparib - EMEA-002269-PIP01-17-M03

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

Day 30 discussion

Action: For discussion

Oncology

3.3.12. Selumetinib - Orphan - EMEA-001585-PIP01-13-M06

AstraZeneca AB; Treatment of melanoma / Treatment of neurofibromatosis type 1 / Treatment of thyroid cancer

Day 30 discussion

Action: For discussion

Oncology

3.3.13. Berotralstat - EMEA-002449-PIP02-18-M02

BioCryst Ireland Limited; Treatment of hereditary angioedema

Day 30 discussion

Action: For discussion

Pneumology - Allergology

3.3.14. Glycopyrronium bromide / formoterol fumarate dihydrate / beclometasone dipropionate - EMEA-001875-PIP02-18-M04

Chiesi Farmaceutici S.p.A.; Treatment of asthma

Day 30 discussion

Action: For discussion

Pneumology - Allergology

3.3.15. Mometasone (furoate) / glycopyrronium bromide / indacaterol - EMEA-001812-PIP01-15-M03

Novartis Europharm Limited; Treatment of asthma

Day 30 discussion

Action: For discussion

Pneumology - Allergology

3.3.16. Atrasentan - Orphan - EMEA-001666-PIP02-21-M01

Chinook Therapeutics, Inc.; Treatment of IgA nephropathy

Day 30 discussion

Action: For discussion

Uro-nephrology

3.3.17. Influenza virus surface antigens (haemagglutinin and neuraminidase) of strain B (Victoria lineage) / influenza virus surface antigens (haemagglutinin and neuraminidase) of strain B (Yamagata lineage) / influenza virus surface antigens (haemagglutinin and neuraminidase) of strain A (H3N2) / influenza virus surface antigens (haemagglutinin and neuraminidase) of strain A (H1N1) - EMEA-002068-PIP01-16-M05

Seqirus Netherlands; Prevention of influenza

Day 30 discussion

Action: For discussion

Vaccines

3.3.18. Live, attenuated, dengue virus, serotype 4 (DENV4) / live, attenuated, dengue virus, serotype 3 (DENV3) / live, attenuated, chimeric dengue virus, serotype 2 (DENV2) / live, attenuated, dengue virus, serotype 1 (DENV1) - EMEA-002999-PIP01-21-M01

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

Day 30 discussion

Action: For discussion

Vaccines

3.3.19. Multivalent pneumococcal polysaccharide conjugate to carrier protein - EMEA-002780-PIP02-20-M01

Sanofi Pasteur; Prevention of disease caused by *Streptococcus pneumoniae*

Day 30 discussion

Action: For discussion

Vaccines

- 3.3.20. Recombinant influenza hemagglutinin-strain B (Yamagata lineage) / recombinant influenza hemagglutinin-strain B (Victoria lineage) / recombinant influenza hemagglutinin-strain A (H3N2 subtype) / recombinant influenza hemagglutinin-strain A (H1N1 subtype) - EMEA-002418-PIP01-18-M03
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Sanofi Pasteur; Prevention of influenza infection

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 26 February 2024 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. EMEA-15-2023

Boehringer Ingelheim International GmbH; All classes of medicinal products for treatment of age-related macular degeneration and diabetic macular oedema / Treatment of geographic atrophy (GA) secondary to age-related macular degeneration (AMD)

Action: For adoption

6.1.2. Zanidatamab - EMEA-01-2024

Jazz Pharmaceuticals Ireland Limited; The class of Her- / epidermal growth factor-receptor antibody medicinal products for treatment of breast malignant neoplasms

Action: For adoption

6.1.3. Tasquinimod – EMEA-16-2023

Active Biotech AB; The class of ribonucleotide reductase beta-2 inhibitor medicinal products for treatment of myeloproliferative neoplasms; the class of primarily alkylating medicinal products for treatment of myeloproliferative neoplasms and mature B, T and NK cell neoplasms; the class of immunomodulatory cytokine medicinal products for treatment of neuroendocrine malignant neoplasms, skin malignant neoplasms, myeloproliferative neoplasms and mature B, T and NK cell neoplasms / Treatment of myelofibrosis

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. Quizartinib - EMEA-001821-PIP01-15-M07

Daiichi Sankyo Europe GmbH; Treatment of acute myeloid leukaemia

Action: For adoption

7.1.2. Abelacimab - EMEA-003017-PIP01-21

Anthos Therapeutics, Inc.; Prevention of thromboembolic events / Treatment of thromboembolic events

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) – Leuven, Belgium, 16-17 May 2024

Update on the SRLM meeting to be held in Leuven during the upcoming Belgium Presidency

PDCO member: Karen van Malderen

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

PDCO member: Karen van Malderen

Action: For information

9.3.2. Paediatric Formulation Operational Expert Group (PFOEG)

PDCO member: Brian Aylward (*ad interim*)

Action: For information

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

Summary report - PCWP-HCPWP - 14-15 November 2023

Draft agenda - PCWP-HCPWP Joint meeting - 27-28 February 2024

Action: For information

9.3.4. Overview of Innovation Task Force (ITF) activities for the year 2023

Action: For information

9.3.5. Upcoming Innovation Task Force (ITF) meetings

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

9.4.2. Biological Quality European Specialised Expert Community (ESEC)

Call for nominations for members

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

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. Main PDCO approaches for the condition pulmonary arterial hypertension

Action: For information

10.2. Guideline on allergen on small populations

Action: For adoption

10.3. EU Network Training Centre (EU NTC): Paediatric curriculum and feedback on Learning & Development toolkit

Update on the Paediatric Curriculum – call for action

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. 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.

For a list of acronyms and abbreviations, see:

[Abbreviations used in EMA scientific committees & CMD documents and in relation to EMA's regulatory activities](#)

More detailed information on the above terms can be found on the EMA website: www.ema.europa.eu/