



EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

19 July 2024
EMA/PDCO/321578/2024
Human Medicines Division

Paediatric Committee (PDCO)

Draft Agenda for the meeting on 23-26 July 2024

Chair: Brian Aylward – Vice-Chair: Sylvie Benchetrit

23 July 2024, 14:00 - 19:00, Virtual meeting

24 July 2024, 08:30 - 19:00, Virtual meeting

25 July 2024, 08:30 - 19:00, Virtual meeting

26 July 2024, 08:30 - 13:00, Virtual 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 23-26 July 2024. See July 2024 PDCO minutes (to be published post September 2024 PDCO meeting).

1.2. Adoption of agenda

PDCO agenda for 23-26 July 2024.

1.3. Adoption of the minutes

PDCO minutes for 25-28 June 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. Seralutinib - Orphan - EMEA-002972-PIP02-23

Gossamer Bio 002 Limited; Treatment of pulmonary arterial hypertension

Day 120 opinion

Action: For adoption

Cardiovascular Diseases

2.1.2. Pegtibatnase - Orphan - EMEA-003518-PIP01-23

Travere Therapeutics Ireland Limited; Treatment of classical homocystinuria

Day 120 opinion

Action: For adoption

Endocrinology-Gynaecology-Fertility-Metabolism

2.1.3. Efinopegdutide - EMEA-003549-PIP01-23

Treatment of non-alcoholic steatohepatitis

Day 120 opinion

Action: For adoption

Gastroenterology-Hepatology

2.1.4. [Recombinant humanised IgG1, kappa light chain, long-acting monoclonal antibody - EMEA-003510-PIP01-23](#)

Prevention of hereditary angioedema attacks

Day 120 opinion

Action: For adoption

Haematology-Hemostaseology

2.1.5. [Iptacopan - EMEA-002705-PIP06-23](#)

Treatment of myasthenia gravis

Day 120 opinion

Action: For adoption

Neurology

2.1.6. [Adeno-associated viral vector serotype 8 containing the 3' human otoferlin coding sequence / adeno-associated viral vector serotype 8 containing the 5' human otoferlin coding sequence - Orphan - EMEA-003524-PIP01-23](#)

Sensorion SA; Treatment of otoferlin gene-mediated hearing loss

Day 120 opinion

Action: For adoption

Other

2.1.7. [Mannose-1-phosphate - Orphan - EMEA-003460-PIP01-23](#)

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

Day 120 opinion

Action: For adoption

Other

2.1.8. [Retatrutide - EMEA-003258-PIP02-23](#)

Treatment of obesity

Day 120 opinion

Action: For adoption

Other

2.1.9. Riloncept - Orphan - EMEA-003571-PIP01-23

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

Day 120 opinion

Action: For adoption

Other / Cardiovascular Diseases

2.1.10. EMEA-003632-PIP01-24

Treatment of heart failure

Day 60 opinion

Action: For adoption

Cardiovascular Diseases

2.1.11. Zerlasiran - EMEA-003627-PIP01-24

Prevention of cardiovascular events

Day 60 opinion

Action: For adoption

Cardiovascular Diseases

2.1.12. Rememulgene arelactibac - EMEA-003638-PIP01-24

Treatment of diabetic foot ulcer / Treatment of venous leg ulcer

Day 60 opinion

Action: For adoption

Dermatology

2.1.13. Aldesleukin - Orphan - EMEA-001556-PIP02-24

ILTOO Pharma SAS; Treatment of amyotrophic lateral sclerosis (ALS)

Day 60 opinion

Action: For adoption

Neurology

2.1.14. Anitocabtagene autoleucel - EMEA-003628-PIP01-24

Treatment of multiple myeloma

Day 60 opinion

Action: For adoption

Oncology

2.1.15. Tafasitamab - Orphan - EMEA-002499-PIP03-24

Incyte Biosciences Distribution B.V; Treatment of mature B-cell neoplasms [excluding diffuse large B-cell lymphoma (DLBCL)]

Day 60 opinion

Action: For adoption

Oncology

2.1.16. Rupatadine / montelukast - EMEA-003629-PIP01-24

Treatment of allergic rhinitis

Day 60 opinion

Action: For adoption

Oto-rhino-laryngology

2.2. Opinions on Compliance Check

2.2.1. Emtricitabine / tenofovir alafenamide - EMEA-C1-001577-PIP02-14-M05

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

Day 60 letter

Action: For adoption

Infectious Diseases

2.2.2. Palbociclib - EMEA-C-002146-PIP01-17-M04

Pfizer Europe MA EEIG; Treatment of Ewing sarcoma

Day 60 opinion

Action: For adoption

Oncology

2.2.3. Avatrombopag - EMEA-C-001136-PIP01-11-M03

Swedish Orphan Biovitrum (AB); Treatment of idiopathic thrombocytopenic purpura

Day 30 opinion

Action: For adoption

Haematology-Hemostaseology

2.2.4. Eltrombopag - EMEA-C-000170-PIP03-13-M04

Novartis Europharm Limited; Treatment of aplastic anaemia

Day 30 opinion

Action: For adoption

Haematology-Hemostaseology

2.3. Opinions on Modification of an Agreed Paediatric Investigation Plan

2.3.1. Fenebrutinib - EMEA-002349-PIP03-20-M01

Roche Registration GmbH; Treatment of multiple sclerosis

Day 60 opinion

Action: For adoption

Neurology

2.3.2. Ambrisentan - Orphan - EMEA-000434-PIP01-08-M10

GlaxoSmithKline (Ireland) Limited; Treatment of pulmonary arterial hypertension

Day 60 opinion

Action: For adoption

Cardiovascular Diseases

2.3.3. Spesolimab - EMEA-002475-PIP03-22-M01

Boehringer Ingelheim International GmbH; Treatment of Netherton syndrome

Day 60 opinion

Action: For adoption

Dermatology

2.3.4. Macimorelin acetate - EMEA-001988-PIP01-16-M02

Atnahs Pharma Netherlands B. V.; Diagnosis of growth hormone deficiency

Day 60 opinion

Action: For adoption

Diagnostic

2.3.5. Osilodrostat - Orphan - EMEA-000315-PIP02-15-M04

Recordati Rare Diseases SARL; Treatment of adrenal cortical hyperfunction

Day 60 opinion

Action: For adoption

Endocrinology-Gynaecology-Fertility-Metabolism

2.3.6. Apremilast - EMEA-000715-PIP02-11-M08

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

2.3.7. Apremilast - EMEA-000715-PIP05-13-M06

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

Day 60 opinion

Action: For adoption

Immunology-Rheumatology-Transplantation

2.3.8. Deucravacitinib - EMEA-002350-PIP02-20-M01

Bristol-Myers Squibb Pharma EEIG; Treatment of chronic idiopathic arthritis (including rheumatoid arthritis, psoriatic arthritis, ankylosing spondylitis and juvenile idiopathic arthritis)

Day 60 opinion

Action: For adoption

Immunology-Rheumatology-Transplantation

2.3.9. Human plasma derived C1-inhibitor (OCTA-C1-INH) - EMEA-002818-PIP01-20-M01

Octapharma Pharmazeutika Produktionsges. m.b.H; Treatment of hereditary angioedema (HAE)

Day 60 opinion

Action: For adoption

Immunology-Rheumatology-Transplantation

2.3.10. Cefiderocol - EMEA-002133-PIP01-17-M04

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

Day 60 opinion

Action: For adoption

Infectious Diseases

2.3.11. Rezafungin acetate - Orphan - EMEA-002319-PIP01-17-M03

Mundipharma GmbH; Treatment of invasive candidiasis

Day 60 opinion

Action: For adoption

Infectious Diseases

2.3.12. Sotrovimab - EMEA-002899-PIP01-20-M03

GlaxoSmithKline Trading Services Ltd; Treatment of coronavirus disease 2019 (COVID-19)

Day 60 opinion

Action: For adoption

Infectious Diseases

2.3.13. Tecovirimat monohydrate - Orphan - EMEA-001205-PIP02-19-M03

SIGA Technologies, Inc.; Treatment of the following viral infections in adults and children with body weight at least 13 kg: smallpox, monkeypox, cowpox. Also indicated to treat complications due to replication of vaccinia virus following vaccination against smallpox in adults and children with body weight at least 13 kg.

Day 60 opinion

Action: For adoption

Infectious Diseases

2.3.14. Lasmiditan - EMEA-002166-PIP01-17-M08

Eli Lilly and Company Ltd; Treatment of migraine headaches

Day 60 opinion

Action: For adoption

Neurology

2.3.15. Rimegepant - EMEA-002812-PIP02-20-M03

Pfizer Europe MA EEIG; Treatment of migraine headaches

Day 60 opinion

Action: For adoption

Neurology

2.3.16. Tasimelteon - Orphan - EMEA-001531-PIP01-13-M06

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

Day 60 opinion

Action: For adoption

Neurology

2.3.17. Vesleteplirsén - EMEA-003305-PIP01-22-M01

Sarepta Therapeutics Ireland; Treatment of Duchenne muscular dystrophy

Day 60 opinion

Action: For adoption

Neurology

2.3.18. Avelumab - EMEA-001849-PIP02-15-M05

Merck Healthcare KGaA; Treatment of malignant neoplasms of the central nervous system

Day 60 opinion

Action: For adoption

Oncology

2.3.19. Daratumumab - Orphan - EMEA-002152-PIP01-17-M04

Janssen-Cilag International NV; Treatment of lymphoid malignancies (except mature B-cell neoplasms)

Day 60 opinion

Action: For adoption

Oncology

2.3.20. Gemtuzumab ozogamicin - Orphan - EMEA-001733-PIP02-15-M03

Pfizer Europe MA EEIG; Treatment of acute myeloid leukaemia

Day 60 opinion

Action: For adoption

Oncology

2.3.21. [Zamtocabtagene autoleucel - Orphan - EMEA-003009-PIP01-21-M01](#)

Miltenyi Biomedicine GmbH; Treatment of mature B-cell neoplasms

Day 60 opinion

Action: For adoption

Oncology

2.3.22. [Patiromer sorbitex calcium - EMEA-001720-PIP01-14-M04](#)

Vifor Fresenius Medical Care Renal Pharma France; Treatment of hyperkalaemia

Day 60 opinion

Action: For adoption

Other

2.3.23. [Iptacopan - Orphan - EMEA-002705-PIP03-20-M01](#)

Novartis Europharm Limited; Treatment of paroxysmal nocturnal haemoglobinuria (PNH)

Day 60 opinion

Action: For adoption

Other / Haematology-Hemostaseology

2.3.24. [Ebola Zaire vaccine \(rVSVΔG-ZEBOV-GP, live\) - EMEA-001786-PIP01-15-M04](#)

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

Day 60 opinion

Action: For adoption

Vaccines

2.3.25. [Multivalent pneumococcal polysaccharide conjugate to carrier protein - EMEA-002780-PIP02-20-M02](#)

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

Day 60 opinion

Action: For adoption

Vaccines

2.3.26. Recombinant varicella zoster virus glycoprotein E - EMEA-001426-PIP01-13-M03

GlaxoSmithKline Biologicals; Prevention of varicella zoster virus (VZV) reactivation

Day 60 opinion

Action: For adoption

Vaccines

2.3.27. Leriglitazone - Orphan - EMEA-002106-PIP01-16-M03

Minoryx Therapeutics S.L.; Treatment of adrenoleukodystrophy

Day 30 opinion

Action: For adoption

Neurology

2.3.28. Etrasimod L-arginine - EMEA-002713-PIP02-21-M02

Pfizer Europe MA EEIG; Treatment of Crohn's disease

Day 30 opinion

Action: For adoption

Gastroenterology-Hepatology

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. Avapritinib - EMEA-C2-002358-PIP02-18-M03

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

Day 30 letter

Action: For information

Oncology

2.7.2. Danicopan - EMEA-C2-002310-PIP01-17-M01

Alexion Europe SAS; Treatment of paroxysmal nocturnal haemoglobinuria

Day 30 letter

Action: For information

Haematology-Hemostaseology

2.7.3. Mavorixafor - EMEA-C1-002490-PIP01-18

X4 Pharmaceuticals (Austria) GmbH; Treatment of warts, hypogammaglobulinemia, infections and myelokathexis (WHIM) syndrome

Day 30 letter

Action: For information

Immunology-Rheumatology-Transplantation

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

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

Day 90 discussion

Action: For discussion

Dermatology / Haematology-Hemostaseology

3.1.2. EMEA-003531-PIP01-23

Treatment of hyperphenylalaninemia

Day 90 discussion

Action: For discussion

Endocrinology-Gynaecology-Fertility-Metabolism

3.1.3. Venglustat - Orphan - EMEA-001716-PIP08-23

Sanofi B.V.; Treatment of Fabry disease

Day 90 discussion

Action: For discussion

Endocrinology-Gynaecology-Fertility-Metabolism

3.1.4. Hydroxycarbamide - EMEA-003388-PIP01-23

Treatment of sickle cell disease

Day 90 discussion

Action: For discussion

Haematology-Hemostaseology

3.1.5. Belumosudil - Orphan - EMEA-003425-PIP02-23

Sanofi Winthrop Industrie; Treatment of chronic lung allograft dysfunction

Day 90 discussion

Action: For discussion

Immunology-Rheumatology-Transplantation

3.1.6. Proline derivative - EMEA-003440-PIP01-23

Treatment of type 1 interferonopathies

Day 90 discussion

Action: For discussion

Immunology-Rheumatology-Transplantation

3.1.7. Dazukibart - Orphan - EMEA-003089-PIP02-23

Pfizer Europe MA EEIG; Treatment of idiopathic inflammatory myopathy

Day 90 discussion

Action: For discussion

Immunology-Rheumatology-Transplantation / Dermatology

3.1.8. 3,3-dimethyl-N-(6-methyl-5-[[2-(1-methyl-1H-pyrazol-4-yl)pyridine-4-yl]oxy}pyridine-2-yl)-2-oxopyrrolidine-1-carboxamide hydrochloride hydrate - Orphan - EMEA-003495-PIP02-24

Abbisko Therapeutics., Co., Ltd.; Treatment of tenosynovial giant cell tumours

Day 90 discussion

Action: For discussion

Oncology

3.1.9. Idroxioleic acid, sodium - Orphan - EMEA-003565-PIP01-23

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

Day 90 discussion

Action: For discussion

Oncology

3.1.10. Recombinant varicella zoster virus glycoprotein E adjuvanted - EMEA-003526-PIP01-23

Prevention of herpes zoster

Day 90 discussion

Action: For discussion

Vaccines

3.1.11. Adeno-associated virus serotype 9 vector containing the human LAMP2 isoform B transgene - Orphan - EMEA-003639-PIP01-24

Rocket Pharmaceuticals, B.V.; Treatment of Danon disease

Day 60 discussion

Action: For discussion

Cardiovascular Diseases

3.1.12. EMEA-003633-PIP01-24

Treatment of psoriasis

Day 60 discussion

Action: For discussion

Dermatology

3.1.13. Dasiglucagon - Orphan - EMEA-002233-PIP03-24

Zealand Pharma A/S; Treatment of congenital hyperinsulinism

Day 60 discussion

Action: For discussion

Endocrinology-Gynaecology-Fertility-Metabolism

3.1.14. Etavopivat - Orphan - EMEA-002924-PIP03-24

Novo Nordisk A/S; Treatment of thalassaemia

Day 60 discussion

Action: For discussion

Haematology-Hemostaseology

3.1.15. Sonelokimab - EMEA-002568-PIP03-24

Treatment of chronic idiopathic arthritis (including rheumatoid arthritis, spondyloarthritis, psoriatic arthritis, and juvenile idiopathic arthritis)

Day 60 discussion

Action: For discussion

Immunology-Rheumatology-Transplantation

3.1.16. Heterologous intestinal microbiota from healthy donors - EMEA-003635-PIP01-24

Treatment of *Clostridioides difficile* infection (CDI)

Day 60 discussion

Action: For discussion

Infectious Diseases

3.1.17. EMEA-003602-PIP01-24

Treatment of tuberculosis

Day 60 discussion

Action: For discussion

Infectious Diseases

3.1.18. Adeno-associated viral vector serotype 9 containing the human MECP2 gene - Orphan - EMEA-003634-PIP01-24

Taysha Gene Therapies, Inc.; Treatment of Rett syndrome

Day 60 discussion

Action: For discussion

Neurology

3.1.19. [H-Arg-Pro-Lys-Pro-Gln-Gln-Phe-2Thi-Gly-Leu-Met\(O₂\)-NH₂-DOTA-225-Actinium - Orphan - EMEA-003630-PIP01-24](#)

NovaCurie GmbH; Treatment of low-grade glioma

Day 60 discussion

Action: For discussion

Neurology

3.1.20. [EMEA-003631-PIP01-24](#)

Treatment of all conditions included in the category of malignant neoplasms (except haematopoietic and lymphoid tissue) whose treatment may include DNA-damaging agents

Day 60 discussion

Action: For discussion

Oncology

3.1.21. [Golcadomide - Orphan - EMEA-003161-PIP02-24](#)

Bristol-Myers Squibb Pharma EEIG; Treatment of mature B-cell neoplasms

Day 60 discussion

Action: For discussion

Oncology

3.1.22. [Esonadogene imvoparvovec - Orphan - EMEA-003626-PIP01-24](#)

Neurophth Therapeutics, Inc.; Treatment of Leber's hereditary optic neuropathy

Day 60 discussion

Action: For discussion

Ophthalmology

3.1.23. [Tacrolimus - EMEA-003625-PIP01-24](#)

Treatment of vernal keratoconjunctivitis

Day 60 discussion

Action: For discussion

Ophthalmology

3.1.24. Human metapneumovirus, virus-like particle / human respiratory syncytial virus, virus-like particle - EMEA-003636-PIP01-24

Prevention of lower respiratory tract disease caused by respiratory syncytial virus or human metapneumovirus

Day 60 discussion

Action: For discussion

Vaccines

3.1.25. mRNA-based vaccine consisting of 3 mRNAs encoding the VP1 capsid protein from NoV genotypes, GII.4, GII.3 and GI.3 - EMEA-003637-PIP01-24

Prevention of norovirus acute gastroenteritis

Day 60 discussion

Action: For discussion

Vaccines

3.1.26. Zirconium (⁸⁹Zr) girentuximab senvedoxam - EMEA-002482-PIP01-24

Diagnostic use for indetermined renal masses (IDRMs) detected by conventional imaging

Day 30 discussion

Action: For discussion

Diagnostic / Oncology / Uro-nephrology

3.1.27. Dexfandrostat phosphate - EMEA-003642-PIP01-24

Treatment of primary aldosteronism

Day 30 discussion

Action: For discussion

Endocrinology-Gynaecology-Fertility-Metabolism / Cardiovascular Diseases

3.1.28. EMEA-003652-PIP01-24

Treatment of non-cirrhotic metabolic dysfunction-associated steatohepatitis (MASH) with fibrosis for homozygous carriers of the PNPLA3 rs738409 148M risk allele / cirrhotic metabolic dysfunction-associated steatohepatitis (MASH) for homozygous carriers of the PNPLA3 rs738409 148M risk allele

Day 30 discussion

Action: For discussion

Gastroenterology-Hepatology

[3.1.29. Duvakitug - EMEA-003647-PIP01-24](#)

Treatment of ulcerative colitis

Day 30 discussion

Action: For discussion

Gastroenterology-Hepatology

[3.1.30. Autologous CD34+ haematopoietic stem cells transduced ex vivo with a lentiviral vector encoding the codon-optimised version of PKLR gene - Orphan - EMEA-003650-PIP01-24](#)

Rocket Pharmaceuticals, B.V.; Treatment of pyruvate kinase deficiency (PKD)

Day 30 discussion

Action: For discussion

Haematology-Hemostaseology

[3.1.31. Riliprubart - Orphan - EMEA-002903-PIP04-24](#)

Sanofi B.V.; Treatment of antibody-mediated rejection (AMR) in kidney transplant recipients

Day 30 discussion

Action: For discussion

Immunology-Rheumatology-Transplantation / Haematology-Hemostaseology / Neurology

[3.1.32. Lenacapavir sodium - EMEA-002740-PIP03-24](#)

Prevention of human immunodeficiency virus (HIV-1) infection

Day 30 discussion

Action: For discussion

Infectious Diseases

[3.1.33. Lenacapavir / islatravir - EMEA-003655-PIP01-24](#)

Treatment of human immunodeficiency virus (HIV-1) infection

Day 30 discussion

Action: For discussion

Infectious Diseases

3.1.34. Ruzasvir / bemnifosbuvir - EMEA-003653-PIP01-24

Treatment of chronic hepatitis C

Day 30 discussion

Action: For discussion

Infectious Diseases

3.1.35. Zosurabalpin - EMEA-003645-PIP01-24

Treatment of infections due to ABC organisms

Day 30 discussion

Action: For discussion

Infectious Diseases

3.1.36. Probenecid - EMEA-003654-PIP01-24

Prevention of epilepsy in focal cortical dysplasia

Day 30 discussion

Action: For discussion

Neurology

3.1.37. Recombinant human progranulin fused to an Fc fragment engineered to contain a human transferrin receptor 1 binding domain - EMEA-003643-PIP01-24

Treatment of frontotemporal dementia

Day 30 discussion

Action: For discussion

Neurology

3.1.38. Remibrutinib - EMEA-002582-PIP04-24

Treatment of myasthenia gravis

Day 30 discussion

Action: For discussion

Neurology

3.1.39. ALK inhibitor - EMEA-003648-PIP01-24

Treatment of malignant lung neoplasms

Day 30 discussion

Action: For discussion

Oncology

3.1.40. Plinabulin - EMEA-003646-PIP01-24

Treatment of stage IIIb and IV non-small cell lung cancer (NSCLC)

Day 30 discussion

Action: For discussion

Oncology

3.1.41. Zidesamtinib - EMEA-003649-PIP01-24

Treatment of malignant lung neoplasms

Day 30 discussion

Action: For discussion

Oncology

3.1.42. Zipalertinib - EMEA-003656-PIP01-24

Treatment of non-small cell lung cancer

Day 30 discussion

Action: For discussion

Oncology

3.1.43. Lunsekimig - EMEA-003644-PIP01-24

Treatment of asthma

Day 30 discussion

Action: For discussion

Pneumology - Allergology

3.1.44. Alogabat - EMEA-003651-PIP01-24

Treatment of autism spectrum disorder (ASD)

Day 30 discussion

Action: For discussion

Psychiatry

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. Efinaconazole - EMEA-C-001627-PIP01-14-M03

Almirall S.A.; Treatment of onychomycosis

Day 30 discussion

Action: For discussion

Dermatology

3.2.2. Metreleptin - EMEA-C3-001701-PIP01-14-M02

Amryt Pharmaceuticals DAC; Treatment of lipodystrophy

Day 30 discussion

Action: For discussion

Gastroenterology-Hepatology

3.2.3. Belumosudil mesylate - EMEA-C1-003425-PIP01-23

Sanofi Winthrop Industrie; Treatment of graft versus host disease (GVHD)

Day 30 discussion

Action: For discussion

Immunology-Rheumatology-Transplantation

3.2.4. Ixekizumab - EMEA-C-001050-PIP02-18-M02

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

Day 30 discussion

Action: For discussion

Immunology-Rheumatology-Transplantation

3.2.5. Dalbavancin hydrochloride - EMEA-C-000016-PIP01-07-M09

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

Day 30 discussion

Action: For discussion

Infectious Diseases

3.2.6. Cannabidiol - EMEA-C4-001964-PIP01-16-M04

Jazz Pharmaceutical Ireland Ltd.; Treatment of seizures associated with Dravet syndrome

Day 30 discussion

Action: For discussion

Neurology

3.2.7. Influenza vaccine (surface antigen, inactivated, prepared in cell cultures) - EMEA-C-002068-PIP01-16-M05

Seqirus Netherlands B.V.; Prevention of influenza

Day 30 discussion

Action: For discussion

Vaccines

3.2.8. Mirdametinib - EMEA-C1-003525-PIP01-23

SpringWorks Therapeutics Ireland Ltd; Treatment of neurofibromatosis type 1

Day 30 discussion

Action: For discussion

Other

3.3. Discussions on Modification of an Agreed Paediatric Investigation Plan

3.3.1. Dupilumab - EMEA-001501-PIP07-20-M02

Sanofi Winthrop Industrie; Treatment of chronic spontaneous urticaria

Day 30 discussion

Action: For discussion

Dermatology

3.3.2. Lebrikizumab - EMEA-002536-PIP01-18-M04

Eli Lilly and Company Limited; Treatment of atopic dermatitis

Day 30 discussion

Action: For discussion

Dermatology

3.3.3. Romosozumab - EMEA-001075-PIP04-15-M07

UCB Pharma S.A.; Treatment of osteoporosis

Day 30 discussion

Action: For discussion

Endocrinology-Gynaecology-Fertility-Metabolism

3.3.4. Mepolizumab - EMEA-000069-PIP01-07-M09

GSK Trading Services Limited; Treatment of hypereosinophilic syndrome

Day 30 discussion

Action: For discussion

Haematology-Hemostaseology

3.3.5. Recombinant human A disintegrin and metalloprotease with thrombospondin type-1 motifs 13 (rADAMTS13) - Orphan - EMEA-001160-PIP01-11-M05

Takeda Pharmaceuticals International AG; Treatment of thrombotic thrombocytopenic purpura

Day 30 discussion

Action: For discussion

Haematology-Hemostaseology

3.3.6. Belatacept - EMEA-000157-PIP01-07-M06

Bristol-Myers Squibb Pharma EEIG; Prevention of rejection of transplanted kidney

Day 30 discussion

Action: For discussion

Immunology-Rheumatology-Transplantation

3.3.7. Upadacitinib - EMEA-001741-PIP01-14-M08

AbbVie Ltd; Treatment of chronic idiopathic arthritis

Day 30 discussion

Action: For discussion

Immunology-Rheumatology-Transplantation

3.3.8. Ustekinumab - EMEA-000311-PIP03-11-M07

Janssen-Cilag International NV; Treatment of chronic idiopathic arthritis (including rheumatoid arthritis, psoriatic arthritis, ankylosing spondylarthritis and juvenile idiopathic

arthritis)

Day 30 discussion

Action: For discussion

Immunology-Rheumatology-Transplantation

3.3.9. Ibrexafungerp citrate - Orphan - EMEA-002535-PIP04-21-M02

SCYNEXIS, Inc.; Prevention of recurrent vulvovaginal candidiasis / Treatment of vulvovaginal candidiasis

Day 30 discussion

Action: For discussion

Infectious Diseases

3.3.10. Posaconazole - EMEA-000468-PIP02-12-M09

MSD Europe Belgium SRL; Prevention of invasive fungal infections / Treatment of invasive fungal infections

Day 30 discussion

Action: For discussion

Infectious Diseases

3.3.11. Tazobactam / ceftolozane - EMEA-001142-PIP02-16-M02

MSD Europe Belgium SRL; Treatment of pneumonia

Day 30 discussion

Action: For discussion

Infectious Diseases

3.3.12. Ganaxolone - Orphan - EMEA-002341-PIP01-18-M03

Marinus Pharmaceuticals Inc.; Treatment of cyclin-dependent kinase-like 5 (CDKL5) deficiency disorder

Day 30 discussion

Action: For discussion

Neurology

3.3.13. Pridopidine (hydrochloride) - Orphan - EMEA-003174-PIP01-21-M02

Prilenia Therapeutics B.V.; Treatment of Huntington disease (HD)

Day 30 discussion

Action: For discussion

Neurology

3.3.14. Vamorolone - Orphan - EMEA-001794-PIP02-16-M09

Santhera Pharmaceuticals (Deutschland) GmbH; Treatment of Duchenne muscular dystrophy

Day 30 discussion

Action: For discussion

Neurology

3.3.15. Avapritinib - Orphan - EMEA-002358-PIP02-18-M04

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

Day 30 discussion

Action: For discussion

Oncology

3.3.16. Daunorubicin / cytarabine - Orphan - EMEA-001858-PIP02-16-M04

Jazz Pharmaceuticals Ireland Limited; Treatment of acute myeloid leukaemia

Day 30 discussion

Action: For discussion

Oncology

3.3.17. Fidirsertib - Orphan - EMEA-003133-PIP01-21-M01

Ipsen Pharma; Treatment of fibrodysplasia ossificans progressiva (FOP)

Day 30 discussion

Action: For discussion

Other

3.3.18. Lomitapide (as lomitapide mesylate) - EMEA-001124-PIP01-10-M06

Amryt Pharmaceuticals DAC; Treatment of heterozygous and homozygous familial hypercholesterolaemia

Day 30 discussion

Action: For discussion

Other

3.3.19. Brensocatib - EMEA-002905-PIP01-20-M01

Insmed Netherlands B.V.; Treatment of non-cystic fibrosis bronchiectasis

Day 30 discussion

Action: For discussion

Pneumology - Allergology

3.3.20. Esketamine hydrochloride - EMEA-002772-PIP01-20-M01

Celon Pharma S.A.; Treatment of bipolar depression / Treatment of major depressive disorder

Day 30 discussion

Action: For discussion

Psychiatry

3.3.21. Pegcetacoplan - Orphan - EMEA-002600-PIP03-21-M03

Swedish Orphan Biovitrum AB (publ); Treatment of glomerulonephritis and nephrotic syndrome

Day 30 discussion

Action: For discussion

Uro-nephrology

3.3.22. mRNA encoding for the linked NTD and RBD domains of the spike glycoprotein of SARS-CoV-2 (mRNA-1283) - EMEA-003426-PIP01-23-M02

MODERNA BIOTECH SPAIN, S.L.; Prevention of coronavirus disease 2019 (COVID-19)

Day 30 discussion

Action: For discussion

Vaccines

3.3.23. *Neisseria meningitidis* serogroup W polysaccharide conjugated to tetanus toxoid / *Neisseria meningitidis* serogroup Y polysaccharide conjugated to tetanus toxoid / *Neisseria meningitidis* serogroup C polysaccharide conjugated to tetanus toxoid / *Neisseria meningitidis* serogroup A polysaccharide conjugated to tetanus toxoid - EMEA-001930-PIP01-16-M06

Sanofi Pasteur; Prevention of invasive meningococcal disease

Day 30 discussion

Action: For discussion

Vaccines

3.3.24. Respiratory syncytial virus vaccine (bivalent, recombinant) - EMEA-002795-PIP02-21-M02

Pfizer Europe MA EEIG; Prevention of lower respiratory tract disease caused by respiratory syncytial virus

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 19 August 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. Tezepelumab - EMEA-08-2024

AstraZeneca AB; All classes of medicinal products for the treatment of chronic obstructive

pulmonary disease (COPD) (excluding chronic lung diseases associated with long-term airflow limitation, such as asthma, bronchopulmonary dysplasia, primary cilia dyskinesia, obstructive lung disease related to graft versus-host disease after [bone-marrow] transplantation).

Proposed indication is as an add-on to inhaled maintenance treatment in COPD patients with a history of exacerbations guided by blood eosinophil (EOS) counts.

Action: For adoption

6.1.2. EMA/PE/0000183128

Biogen Netherlands B.V.; Alzheimer's disease

Action: For adoption

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

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

No item

8. Annual reports on deferrals

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

9. Organisational, regulatory and methodological matters

9.1. Mandate and organisation of the PDCO

9.1.1. PDCO membership

Action: For information

9.1.2. Vote by Proxy

Action: For information

9.1.3. Strategic Review and Learning Meeting (SRLM) – Budapest, Hungary, 9-10 October 2024

Update on the SRLM meeting to be held in Budapest during the upcoming Hungarian

Presidency

PDCO member: Adrienn Horváth

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)

No item

9.3.4. 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)

Action: For information

9.5. Cooperation with International Regulators

9.5.1. Paediatric Cluster Teleconference

Action: For information

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

No item

9.7. PDCO work plan

9.7.1. Workplan for 2024

Scope: Progress of the 2024 workplan

PDCO Chair: Brian Aylward

Action: For information

9.8. Planning and reporting

9.8.1. EMA Business Pipeline activity and Horizon scanning

No item

10. Any other business

10.1. ICH E11A on Paediatric Extrapolation

Scope: ICH E11A has been finalised as the comments received during public consultation have been addressed and it was adopted at CHMP PROM in July 2024.

Action: For information

10.2. PDCO Agenda and Time schedule in IRIS

Scope: The PDCO will be shown a brief live demo of the agenda and time schedule in IRIS

Action: For information

11. Breakout sessions

11.1. Internal PDCO Operations

Action: For discussion on Tuesday, 12:00 - 13:00

11.2. Paediatric oncology

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

11.3. Neonatology

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

11.4. 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.

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/