24 March 2023
EMA/PDCO/95095/2023
Human Medicines Division

Paediatric Committee (PDCO)

Draft Agenda for the meeting on 28-31 March 2023

Chair: Brian Aylward – Vice-Chair: Sylvie Benchetrit

28 March 2023, 14:00 - 19:30, room 2C

29 March 2023, 08:30 - 19:30, room 2C

30 March 2023, 08:30 - 19:30, room 2C

31 March 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 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 28-31 March 2023. See March 2023 PDCO minutes (to be published post April 2023 PDCO meeting).

1.2. Adoption of agenda

PDCO agenda for 28-31 March 2023.

1.3. Adoption of the minutes

PDCO minutes for 21-24 February 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. EMEA-002612-PIP02-22

Treatment of sickle cell disease

Day 120 opinion

Action: For adoption

Haematology-Hemostaseology

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

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

Day 120 opinion

Action: For adoption

Immunology-Rheumatology-Transplantation

2.1.3. Albaconazole - EMEA-003279-PIP01-22

Treatment of acute vulvovaginal candidiasis

Day 120 opinion

Action: For adoption

Infectious Diseases

2.1.4. Opelconazole - EMEA-003249-PIP01-22

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

Day 120 opinion

Action: For adoption

Infectious Diseases

Note: Withdrawal request received on 23 March 2023

2.1.5. 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)

Day 120 opinion

Action: For adoption

Neurology

2.1.6. Ocrelizumab - EMEA-000310-PIP05-22

Treatment of multiple sclerosis

Day 120 opinion

Action: For adoption

Neurology

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

Treatment of Hodgkin lymphoma

Day 120 opinion

Action: For adoption

Oncology

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

Treatment of Hodgkin lymphoma

Day 120 opinion

Action: For adoption

Oncology

2.1.9. Aticaprant - EMEA-003251-PIP01-22

Treatment of major depressive disorder

Day 120 opinion

Action: For adoption

Psychiatry

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

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

Day 120 opinion

Action: For adoption

Uro-nephrology

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

Treatment of homozygous familial hypercholesterolaemia (HoFH) / hypercholesterolaemia

Day 60 opinion

Action: For adoption

Cardiovascular Diseases

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

Treatment of hypertension

Day 60 opinion

Action: For adoption

Cardiovascular Diseases

2.1.13. Nipocalimab - Orphan - EMEA-002559-PIP07-22

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

Day 60 opinion

Action: For adoption

Immunology-Rheumatology-Transplantation

2.1.14. Adeno-associated viral vector serotype 9 expressing codon-optimized human GRN gene (LY3884963) - Orphan - EMEA-003374-PIP01-22

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

Day 60 opinion

Action: For adoption

Neurology

2.1.15. EMEA-003364-PIP02-22

Treatment of non-small cell lung cancer

Day 60 opinion

Action: For adoption

Oncology

2.1.16. 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 60 opinion

Action: For adoption

Oncology

2.1.17. 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 60 opinion

Action: For adoption

Oncology

2.1.18. 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 head and neck squamous cell carcinoma (HNSCC) / Treatment of renal cell carcinoma (RCC)

Day 60 opinion

Action: For adoption

Oncology

2.1.19. Ezetimibe / pitavastatin - EMEA-003390-PIP01-23

Treatment of hypercholesterolaemia

Day 30 opinion

Action: For adoption

Cardiovascular Diseases

2.1.20. Thiocolchicoside / diclofenac - EMEA-003339-PIP02-23

Treatment of acute painful muscle spasms / Treatment of musculoskeletal pain

Day 30 opinion

Action: For adoption

Pain

2.1.21. Chlorhexidine / isopropyl - EMEA-001338-PIP02-23

Prevention of infections prior to minor procedures (low-risk invasive procedures)

Day 30 opinion

Action: For adoption

Infectious Diseases

2.2. Opinions on Compliance Check

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

Amryt Pharmaceuticals DAC; Treatment of epidermolysis bullosa

Day 60 opinion

Action: For adoption

Dermatology

Note: Withdrawal request received on 8 March 2023

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

Novartis Europharm Limited; Treatment of aplastic anaemia

Day 60 opinion

Action: For adoption

Haematology-Hemostaseology

Note: Withdrawal request received on 16 March 2023

2.2.3. 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 60 letter

Action: For adoption

Haematology-Hemostaseology

2.2.4. 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 60 opinion

Action: For adoption

Oncology

2.2.5. Larotrectinib - EMEA-C-001971-PIP02-16-M04

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

Day 30 opinion

Action: For adoption

Oncology

2.3. Opinions on Modification of an Agreed Paediatric Investigation Plan

2.3.1. Tralokinumab - EMEA-001900-PIP02-17-M07

LEO Pharma A/S; Treatment of atopic dermatitis

Day 60 opinion

Action: For adoption

Dermatology

2.3.2. Dasiglucagon - EMEA-002233-PIP01-17-M02

Zealand Pharma A/S; Treatment of hypoglycaemia

Day 60 opinion

Action: For adoption

Endocrinology-Gynaecology-Fertility-Metabolism

2.3.3. Elosulfase alfa - Orphan - EMEA-000973-PIP01-10-M04

BioMarin International Limited; Treatment of mucopolysaccharidosis, type IVA (Morquio A syndrome)

Day 60 opinion

Action: For adoption

Endocrinology-Gynaecology-Fertility-Metabolism

2.3.4. Migalastat (hydrochloride) - Orphan - EMEA-001194-PIP01-11-M06

Amicus Therapeutics Europe Limited; Treatment of Fabry disease

Day 60 opinion

Action: For adoption

Endocrinology-Gynaecology-Fertility-Metabolism

2.3.5. Romosozumab - EMEA-001075-PIP04-15-M06

UCB Pharma S.A.; Treatment of osteoporosis

Day 60 opinion

Action: For adoption

Endocrinology-Gynaecology-Fertility-Metabolism

2.3.6. Tolvaptan - EMEA-001231-PIP02-13-M10

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

Day 60 opinion

Action: For adoption

Endocrinology-Gynaecology-Fertility-Metabolism / Uro-nephrology

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

Janssen-Cilag International NV; Treatment of ulcerative colitis

Day 60 opinion

Action: For adoption

Gastroenterology-Hepatology

2.3.8. 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 60 opinion

Action: For adoption

Gastroenterology-Hepatology

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

BioMarin International Limited; Treatment of haemophilia A

Day 60 opinion

Action: For adoption

Haematology-Hemostaseology

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

AstraZeneca AB; Treatment of systemic lupus erythematosis / lupus nephritis

Day 60 opinion

Action: For adoption

Immunology-Rheumatology-Transplantation

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

AbbVie Ltd; Treatment of atopic dermatitis

Day 60 opinion

Action: For adoption

Immunology-Rheumatology-Transplantation / Dermatology

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

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

Day 60 opinion

Action: For adoption

Infectious Diseases

2.3.13. Maribavir - Orphan - EMEA-000353-PIP02-16-M03

Takeda Pharmaceuticals International AG Ireland Branch; Treatment of cytomegalovirus

(CMV) infection

Day 60 opinion

Action: For adoption

Infectious Diseases

2.3.14. Efgartigimod alfa - Orphan - EMEA-002597-PIP05-21-M01

argenx BV; Treatment of myasthenia gravis

Day 60 opinion

Action: For adoption

Neurology

2.3.15. Lasmiditan - EMEA-002166-PIP01-17-M07

Eli Lilly and Company Limited; Treatment of migraine headaches

Day 60 opinion

Action: For adoption

Neurology

2.3.16. Vatiquinone - Orphan - EMEA-001238-PIP03-21-M01

PTC Therapeutics International; Treatment of Friedreich's ataxia

Day 60 opinion

Action: For adoption

Neurology

2.3.17. Binimetinib - EMEA-001454-PIP03-15-M03

Pierre Fabre Médicament; Treatment of melanoma

Day 60 opinion

Action: For adoption

Oncology

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

Pierre Fabre Médicament; Treatment of melanoma

Day 60 opinion

Action: For adoption

Oncology

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

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

Day 60 opinion

Action: For adoption

Oncology

2.3.20. 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 60 opinion

Action: For adoption

Oncology

2.3.21. 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 60 opinion

Action: For adoption

Other

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

Novartis Europharm Limited; Treatment of C3 glomerulopathy

Day 60 opinion

Action: For adoption

Other

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

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

Day 60 opinion

Action: For adoption

Pain

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

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

Day 60 opinion

Action: For adoption

Pneumology - Allergology

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

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

Day 60 opinion

Action: For adoption

Pneumology - Allergology

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

Bayer AG; Treatment of chronic kidney disease

Day 60 opinion

Action: For adoption

Uro-nephrology

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

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

Day 60 opinion

Action: For adoption

Uro-nephrology

2.3.28. 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 60 opinion

Action: For adoption

Vaccines

2.3.29. Cariprazine - EMEA-001652-PIP01-14-M05

Gedeon Richter Plc.; Treatment of schizophrenia

Day 30 opinion

Action: For adoption

Psychiatry

2.3.30. Posaconazole - EMEA-000468-PIP02-12-M08

Merck Sharp & Dohme (Europe), Inc.; Prevention of invasive fungal infections / Treatment of invasive fungal infections

Day 30 opinion

Action: For adoption

Infectious Diseases

2.3.31. Acetyl-L-leucine ((s)-(acetylamino)-4-methylpentanoic acid) - Orphan - EMEA-002796-PIP01-20-M01

IntraBio Ltd.; Treatment of Niemann-Pick disease type C

Day 30 opinion

Action: For adoption

Neurology

2.3.32. Elasomeran / imelasomeran – elasomeran / davesomeran - elasomeran - EMEA-002893-PIP01-20-M03

Moderna Biotech Spain, S.L.; Prevention of coronavirus disease 2019 (COVID-19)

Day 8 opinion

Action: For adoption

Vaccines

2.4. Opinions on Re-examinations

No item

2.5. Opinions on Review of Granted Waivers

No item

2.6. Finalisation and adoption of Opinions

No item

2.7. Partial Compliance Checks completed by EMA

The following partial compliance checks have been identified by the PME coordinator and PDCO rapporteur as not needing to be referred to the PDCO for discussion. The PDCO has been informed in writing.

2.7.1. Lacosamide - EMEA-C2-000402-PIP03-17-M06

UCB Pharma S.A.; Treatment of generalised epilepsy and epileptic syndromes

Day 30 letter

Action: For information

Neurology

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. Insulin human (RDNA) - EMEA-003194-PIP02-22

Treatment of type 2 diabetes mellitus

Day 90 discussion

Action: For discussion

Endocrinology-Gynaecology-Fertility-Metabolism

3.1.2. Crofelemer - Orphan - EMEA-003296-PIP01-22

Napo Therapeutics S.p.A.; Treatment of short bowel syndrome

Day 90 discussion

Action: For discussion

Gastroenterology-Hepatology

3.1.3. Efgartigimod alfa - EMEA-002597-PIP08-22

Treatment of dermatomyositis / Treatment of polymyositis (including antisynthetase syndrome) / Treatment of immune-mediated necrotising myopathy

Day 90 discussion

Action: For discussion

Immunology-Rheumatology-Transplantation

3.1.4. Asunercept - Orphan - EMEA-003201-PIP01-22

Apogenix AG; Treatment of coronavirus disease 2019 (COVID-19)

Day 90 discussion

Action: For discussion

Infectious Diseases

3.1.5. Fosmanogepix - Orphan - EMEA-003280-PIP01-22

Pfizer Europe MA EEIG; Treatment of invasive fungal infections

Day 90 discussion

Action: For discussion

Infectious Diseases

3.1.6. RNA replicase inhibitor - EMEA-003306-PIP01-22

Treatment of coronavirus disease 2019 (COVID-19)

Day 90 discussion

Action: For discussion

Infectious Diseases

3.1.7. Humanised VHH-type bispecific antibody against complement component 5 and serum albumin - EMEA-003302-PIP01-22

Treatment of acetylcholine receptor-antibody positive generalised myasthenia gravis

Day 90 discussion

Action: For discussion

Neurology

3.1.8. Adult differentiated autologous T-cells from peripheral blood, expanded and transduced with a lentivirus to express a chimeric antigen receptor with anti-CD19 specificity (A3B1) conjugated with the co-stimulatory regions 4-1BB and CD3z - EMEA-003264-PIP01-22

Treatment of B-lymphoblastic leukaemia/lymphoma

Day 90 discussion

Action: For discussion

Oncology

3.1.9. Live attenuated respiratory syncytial virus (RSV) - EMEA-003277-PIP01-22

Prevention of respiratory syncytial virus (RSV) diseases

Day 90 discussion

Action: For discussion

Vaccines

3.1.10. Single-stranded 5' capped mRNA encoding the respiratory syncytial virus glycoprotein F stabilized in the prefusion conformation - EMEA-003309-PIP01-22

Prevention of lower respiratory tract illness (LRTI) caused by respiratory syncytial virus (RSV)

Day 90 discussion

Action: For discussion

Vaccines

3.1.11. Upadacitinib - EMEA-001741-PIP08-22

Treatment of hidradenitis suppurativa

Day 60 discussion

Action: For discussion

Dermatology

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

Treatment of hyperphenylalaninemia

Day 60 discussion

Action: For discussion

Endocrinology-Gynaecology-Fertility-Metabolism

3.1.13. Leuprorelin - EMEA-003354-PIP01-22

Treatment of central (gonadotropin-dependent) precocious puberty

Day 60 discussion

Action: For discussion

Endocrinology-Gynaecology-Fertility-Metabolism

3.1.14. Cemdisiran - Orphan - EMEA-003237-PIP02-22

Regeneron Ireland DAC; Treatment of paroxysmal nocturnal haemoglobinuria

Day 60 discussion

Action: For discussion

Haematology-Hemostaseology

3.1.15. Pozelimab - EMEA-003238-PIP02-22

Treatment of paroxysmal nocturnal haemoglobinuria

Day 60 discussion

Action: For discussion

Haematology-Hemostaseology

3.1.16. Axatilimab - EMEA-003385-PIP01-22

Treatment of chronic graft-versus-host-disease

Day 60 discussion

Action: For discussion

Immunology-Rheumatology-Transplantation

3.1.17. Inebilizumab - EMEA-001911-PIP02-22

Treatment of generalised myasthenia gravis

Day 60 discussion

Action: For discussion

Neurology

3.1.18. 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 60 discussion

Action: For discussion

Oncology

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

Treatment of melanoma

Day 60 discussion

Action: For discussion

Oncology

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

Treatment of spinal muscular atrophy

Day 60 discussion

Action: For discussion

Other

3.1.21. EMEA-003319-PIP04-22

Treatment of borderline personality disorder (BPD)

Day 60 discussion

Action: For discussion

Psychiatry

3.1.22. *Neisseria meningitidis* serogroup B Protein-based active substance / recombinant *Neisseria meningitidis* serogroup B protein 3 / recombinant *Neisseria meningitidis* serogroup B protein 2 / recombinant *Neisseria meningitidis* serogroup B protein 1 / *Neisseria meningitidis* group Y polysaccharide conjugated to tetanus toxoid carrier protein / *Neisseria meningitidis* group W-135 polysaccharide conjugated to tetanus toxoid carrier protein / *Neisseria meningitidis* group 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

Day 60 discussion

Action: For discussion

Vaccines

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

Rezolute (Bio) Ireland Limited; Treatment of hyperinsulinemic hypoglycaemia

Day 30 discussion

Action: For discussion

Endocrinology-Gynaecology-Fertility-Metabolism

3.1.24. Inebilizumab - EMEA-001911-PIP03-23

Treatment of immunoglobulin G4-related disease (IgG4-RD)

Day 30 discussion

Action: For discussion

Immunology-Rheumatology-Transplantation

3.1.25. Upadacitinib - EMEA-001741-PIP09-23

Treatment of systemic lupus erythematosus

Day 30 discussion

Action: For discussion

Immunology-Rheumatology-Transplantation

3.1.26. Broadly neutralising anti-HIV human monoclonal antibody - EMEA-003392-PIP01-23

Treatment of human immunodeficiency virus (HIV-1) infection

Day 30 discussion

Action: For discussion

Infectious Diseases

3.1.27. Carbidopa / levodopa - EMEA-003384-PIP02-23

Treatment of Parkinson's disease

Day 30 discussion

Action: For discussion

Neurology

3.1.28. Deutetrabenazine - EMEA-002052-PIP02-23

Treatment of tardive dyskinesia

Day 30 discussion

Action: For discussion

Neurology

3.1.29. Inhibitor of receptor-interacting serine/threonine-protein kinase 1 (RIPK1) - EMEA-003383-PIP02-23

Treatment of amyotrophic lateral sclerosis

Day 30 discussion

Action: For discussion

Neurology

3.1.30. Belzutifan - Orphan - EMEA-002619-PIP02-23

Merck, Sharp & Dohme (Europe) Inc; Treatment of von Hippel-Lindau disease (except von Hippel-Lindau disease associated renal cell carcinoma) / Treatment of neuroendocrine tumours

Day 30 discussion

Action: For discussion

Oncology

3.1.31. EMEA-003260-PIP02-23

Treatment of biliary tract cancer

Day 30 discussion

Action: For discussion

Oncology

3.1.32. Dordaviprone - Orphan - EMEA-003389-PIP01-23

Chimerix IRL Limited; Treatment of high-grade glioma

Day 30 discussion

Action: For discussion

Oncology

3.1.33. (S)-lactic acid - EMEA-003247-PIP02-23

Treatment of degenerative disc disease

Day 30 discussion

Action: For discussion

Other

3.1.34. ALM (Almonds), CAS (Cashews), COD (Codfish), EGG (Egg), HAZ (Hazelnuts), MIL (Milk), PEA (Peanuts), PEC (Pecans), PIS (Pistachios), SAL (Salmon), SES (Sesame Seed), SHR (Shrimp), SOY (Soybeans), WAL (Walnuts), WHE (Wheat) - EMEA-003397-PIP01-23

Treatment of food allergy

Day 30 discussion

Action: For discussion

Other

3.1.35. EMEA-003394-PIP01-23

Treatment of Duchenne/Becker muscular dystrophy

Day 30 discussion

Action: For discussion

Other / Neurology

3.1.36. Salbutamol - EMEA-003398-PIP01-23

Treatment of asthma

Day 30 discussion

Action: For discussion

Pneumology - Allergology

3.1.37. Tanimilast - EMEA-003393-PIP01-23

Treatment of asthma

Day 30 discussion

Action: For discussion

Pneumology - Allergology

3.1.38. Complement factor B antisense oligonucleotide - EMEA-003396-PIP01-23

Treatment of glomerulonephritis and nephrotic syndrome / Treatment of primary IgAN

Day 30 discussion

Action: For discussion

Uro-nephrology

3.1.39. Haematopoietic stem cells and blood progenitors umbilical cord-derived expanded with (1R,4R)-N1-(2-benzyl-7-(2-methyl-2H-tetrazol-5-yl)-9H-pyrimido[4,5-b]indol-4-yl)cyclohexane-1,4-diamine dihydrobromide dihydrate (ECT-001-CB) - Orphan - EMEA-003025-PIP03-23

ExCellThera; Treatment in allogeneic haematopoietic stem cell transplantation

Day 30 discussion

Action: For discussion

Immunology-Rheumatology-Transplantation / Oncology / Haematology-Hemostaseology

3.1.40. Sodium hyaluronate / xylometazoline - EMEA-003387-PIP01-22

Treatment of acute viral rhinosinusitis

Day 30 discussion

Action: For discussion

Oto-rhino-laryngology

3.1.41. Amphotericin B - Orphan - EMEA-003391-PIP01-23

Matinas BioPharma Holdings Inc.; Treatment of cryptococcosis

Day 30 discussion

Action: For discussion

Infectious Diseases

3.1.42. Iodine (131I) apamistamab - Orphan - EMEA-003395-PIP01-23

Immedica Pharma AB; Conditioning treatment prior to haematopoietic stem cell transplantation (HSCT) in malignant neoplasms of haematopoietic and lymphoid tissue

Day 30 discussion

Action: For discussion

Immunology-Rheumatology-Transplantation / Oncology

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. Dry aqueous extract of *Paullinia cupana* seed / liquid ethanolic extract 30 per cent (w/w) of *Allium cepa* fresh bulb and citrus limon fresh fruit / dry hydroethanolic extract of *Theobroma cacao* seed - EMEA-C1-001835-PIP01-15-M05

LEGACY HEALTHCARE (FRANCE)SAS; Treatment of alopecia

Day 30 discussion

Action: For discussion

Dermatology

3.2.2. Efanesoctocog alfa - EMEA-C-002501-PIP01-18-M03

Swedish Orphan Biovitrium; Treatment of haemophilia A

Day 30 discussion

Action: For discussion

Haematology-Hemostaseology

3.2.3. Crizotinib - EMEA-C-001493-PIP03-18-M01

Pfizer Europe MA EEIG; Treatment of inflammatory myofibroblastic tumour / Treatment of anaplastic large cell lymphoma

Day 30 discussion

Action: For discussion

Oncology

3.3. Discussions on Modification of an Agreed Paediatric Investigation Plan

3.3.1. 3,4-Dimethoxy-N-methylbenzohydroxamic acid / deferoxamine mesylate / alpha-ketoglutaric acid / arginine / alanine / glycine / aspartic acid / tryptophan / N-acetyl-histidine (monohydrate) / histidine / calcium chloride (dihydrate) / magnesium chloride (hexahydrate) / potassium chloride / sodium chloride - EMEA-002735-PIP01-19-M01

Dr. Franz Köhler Chemie GmbH; Cardioplegia

Day 30 discussion

Action: For discussion

Cardiovascular Diseases

3.3.2. 3,4-Dimethoxy-N-methylbenzohydroxamic acid / deferoxamine mesylate / alpha-ketoglutaric acid / arginine / alanine / glycine / gspartic acid / tryptophan / N-acetyl-histidine (monohydrate) / histidine / calcium chloride (dihydrate) / magnesium chloride (hexahydrate) / potassium chloride / sodium chloride - EMEA-002735-PIP03-20-M02

Dr. Franz Köhler Chemie GmbH; Heart transplantation

Day 30 discussion

Action: For discussion

Cardiovascular Diseases

3.3.3. Landiolol (hydrochloride) - EMEA-001150-PIP02-13-M05

AOP Orphan Pharmaceuticals GmbH; Treatment of supraventricular arrythmias

Day 30 discussion

Action: For discussion

Cardiovascular Diseases

3.3.4. Brodalumab - EMEA-001089-PIP02-13-M04

LEO Pharma A/S; Treatment of psoriasis

Day 30 discussion

Action: For discussion

Dermatology

3.3.5. Ethinylestradiol / dienogest - EMEA-002229-PIP02-21-M01

Chemo Research; Treatment of polycystic ovary syndrome

Day 30 discussion

Action: For discussion

Endocrinology-Gynaecology-Fertility-Metabolism

3.3.6. Denecimig - EMEA-002762-PIP02-20-M01

Novo Nordisk A/S; Treatment of haemophilia A

Day 30 discussion

Action: For discussion

Haematology-Hemostaseology

3.3.7. Lamivudine (3TC) / abacavir (ABC) / dolutegravir (DTG) - EMEA-001219-PIP01-11-M07

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

Day 30 discussion

Action: For discussion

Infectious Diseases

3.3.8. Regdanvimab - EMEA-002961-PIP01-21-M02

Celltrion Healthcare Hungary Kft.; Treatment of coronavirus disease 2019 (COVID-19)

Day 30 discussion

Action: For discussion

Infectious Diseases

3.3.9. Sotrovimab - EMEA-002899-PIP01-20-M02

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

Day 30 discussion

Action: For discussion

Infectious Diseases

3.3.10. Tenofovir alafenamide / rilpivirine / emtricitabine - EMEA-001679-PIP01-14-M03

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

Day 30 discussion

Action: For discussion

Infectious Diseases

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

Almirall, S.A.; Treatment of onychomycosis (tinea unguium)

Day 30 discussion

Action: For discussion

Infectious Diseases / Dermatology

3.3.12. Cenobamate - EMEA-002563-PIP02-19-M02

Angelini Pharma S.p.A; Treatment of focal onset seizures / Treatment of primary generalised tonic clonic seizures

Day 30 discussion

Action: For discussion

Neurology

3.3.13. Odronextamab - Orphan - EMEA-003149-PIP01-21-M01

Regeneron Ireland DAC; Treatment of mature B cell malignancies

Day 30 discussion

Action: For discussion

Oncology

3.3.14. Ponesimod - EMEA-000798-PIP01-09-M04

Janssen-Cilag International NV; Treatment of multiple sclerosis

Day 30 discussion

Action: For discussion

Other / Neurology

3.3.15. Adsorbed modified allergen extract of a mixture of 50% *Dermatophagoides pteronyssinus* and 50% *Dermatophagoides farinae* - EMEA-000902-PIP01-10-M01

HAL Allergy BV; Treatment of allergic rhinitis/rhino-conjunctivitis

Day 30 discussion

Action: For discussion

Pneumology - Allergology

3.3.16. Bilastine - EMEA-000347-PIP02-16-M05

Faes Farma S.A.; Treatment of allergic conjunctivitis

Day 30 discussion

Action: For discussion

Ophthalmology

3.3.17. Treprostinil - EMEA-000207-PIP01-08-M08

Ferrer Internacional, S.A.; Other secondary hypertension / Primary pulmonary hypertension

Day 30 discussion

Action: For discussion

Cardiovascular Diseases

3.3.18. Ataluren - Orphan - EMEA-000115-PIP01-07-M13

PTC Therapeutics International, Limited; Treatment of dystrophinopathy

Day 30 discussion

Action: For discussion

Neurology

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 March 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

6.1.1. Anti-human hemojuvelin (HJV) humanized monoclonal antibody - EMEA-09-2022

Disc Medicine B.V.; The class of ribonucleotide reductase beta-2 inhibitor medicinal products, the class of primarily alkylating medicinal products and the class of immunomodulatory cytokine medicinal products for the treatment of myeloproliferative neoplasms / treatment of anaemia in myelofibrosis

Action: For adoption

6.1.2. Non-steroidal small molecule androgen receptor pathway inhibitor - EMEA-11-2022

Merck Sharp & Dohme (Europe), Inc; The class of androgen receptor modulator, oestrogen receptor modulator, of growth and sex hormone as well as their releasing or inhibiting factors, and of sex hormone-metabolism modulator medicinal products for the treatment of prostate malignant neoplasms / treatment of patients with metastatic castration-resistant prostate cancer

Action: For adoption

6.1.3. Elinzanetant - EMEA-01-2023

Bayer AG; All classes of medicinal products for treatment of climacteric symptoms associated with decreased oestrogen levels, as occurring at menopause

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. Berotralstat - EMEA-002449-PIP02-18-M01

BioCryst Ireland Limited; Treatment of hereditary angioedema / Prevention of attacks in patients with hereditary angioedema

Action: For adoption

7.1.2. Vericiguat - EMEA-001636-PIP01-14-M03

Bayer AG; Treatment of left ventricular failure

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

Update on the Strategic Review & Learning meeting under the Swedish Presidency of the Council of the EU, to be held on 7-8 June 2023

PDCO member: Sara Vennberg

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. 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)

No item

9.3.4. Scientific Advice Working Party (SAWP)

Proposal for PDCO consultation of SAWP

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. PDCO / Health Technology Assessment (HTA) interaction

Update on current initiatives

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

Q1/2023 Update of the Business Pipeline report for the human scientific committees

Action: For information

10. Any other business

10.1. COVID-19 update

Action: For information

10.2. WHO paediatric activities

WHO experts: Marie Valentin, Martina Penazzato

Action: For information

10.3. Academic pilot for ATMPs

Action: For information

10.4. Upcoming Innovation Task Force (ITF) meetings

Action: For information

10.5. Concept paper on the revision of the Paediatric Addendum on pulmonary arterial hypertension guideline

Action: For discussion

11. Breakout sessions

11.1. Paediatric oncology

Action: For discussion on Wednesday, 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

11.4. Vaccines

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

12. Explanatory notes

The Notes give a brief explanation of relevant agenda items and should be read in conjunction with the agenda.

Paediatric investigation plan (PIP) (*section 2.1 Opinion on PIPs and section 3.1 Discussions on PIPs*)
A paediatric investigation plan (PIP) is a development plan aimed at ensuring that the necessary data are obtained through studies in children, when it is safe to do so, to support the authorisation of a medicine for children. Pharmaceutical companies submit proposals for PIPs to the European Medicines Agency's Paediatric Committee (PDCO). This Committee is responsible for agreeing or refusing the plan.

Compliance checks (*section 2.2 Opinions on Compliance check, section 3.2 Discussions on Compliance check*)
A compliance check may be necessary before any application for marketing authorisation (even for an adult indication) can be considered valid, if there was no deferral for at least one of the studies agreed in the PIP, or after the due date of initiation or completion of a study/measure. The same applies to some regulatory applications for authorised products, as described above.

Modification of an Agreed Paediatric Investigation Plan (*section 2.3 Opinions on Modification of an agreed PIP, section 3.3 Discussions on Modification of an agreed PIP*)
The development plan for a medicine can be modified at a later stage as knowledge increases. Modifications can also be made if the applicant encounters such difficulties with the implementation of a PIP, which render it unworkable or no longer appropriate.
In some cases, studies can be deferred until after the studies in adults have been conducted. This ensures that research in children is done only when it is safe and ethical to do so. Even when studies are deferred, the PIP will include details of the paediatric studies and their timelines.

Class waiver (*section 6 Discussion on the applicability of class waiver*)
As some diseases do not affect children (for example Parkinson's disease), the development of medicines for these diseases should not be performed in children. In these cases, a PIP is not required and it will be waived**.** For more information on the classes of diseases subject to waivers, see class waivers.

Annual reports on deferrals (*section 8*)
If the medicinal product is approved in the EU, annual reports on the deferred measures in the PIP must be submitted to the Agency.

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