07 December 2023
EMA/PDCO/528422/2023
Human Medicines Division

Paediatric Committee (PDCO)

Draft Agenda for the meeting on 12-15 December 2023

Chair: Brian Aylward – Vice-Chair: Sylvie Benchetrit

12 December 2023, 14:00- 19:00, Virtual meeting

13 December 2023, 08:30- 19:00, Virtual meeting

14 December 2023, 08:30- 19:00, Virtual meeting

15 December 2023, 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 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 12-15 December 2023. See November 2023 PDCO minutes (to be published post December 2023 PDCO meeting).

1.2. Adoption of agenda

PDCO agenda for 12-15 December 2023.

1.3. Adoption of the minutes

PDCO minutes for 07-10 November 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. Obicetrapib - EMEA-003438-PIP02-23

Treatment of elevated cholesterol

Day 120 opinion

Action: For adoption

Cardiovascular Diseases

2.1.2. Remibrutinib - EMEA-002582-PIP03-23

Treatment of chronic inducible urticaria

Day 120 opinion

Action: For adoption

Dermatology

2.1.3. Frexalimab - EMEA-002945-PIP03-23

Treatment of type 1 diabetes mellitus

Day 120 opinion

Action: For adoption

Endocrinology-Gynaecology-Fertility-Metabolism

2.1.4. Semaglutide - EMEA-003402-PIP01-23

Treatment of non-alcoholic steatohepatitis (NASH)

Day 120 opinion

Action: For adoption

Gastroenterology-Hepatology

2.1.5. Tarperprumig - Orphan - EMEA-003432-PIP01-23

Alexion Europe SAS; Treatment of sickle cell disease (SCD)

Day 120 opinion

Action: For adoption

Haematology-Hemostaseology

Note: Withdrawal request received on 27 November 2023

2.1.6. EMEA-003271-PIP02-22

Treatment of epilepsy syndromes / Treatment of primary generalised tonic-clonic seizures

Day 120 opinion

Action: For adoption

Neurology

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

Chimerix IRL Limited; Treatment of glioma

Day 120 opinion

Action: For adoption

Oncology

2.1.8. Trotabresib - EMEA-003361-PIP01-22

Treatment of malignant neoplasms of the central nervous system

Day 120 opinion

Action: For adoption

Oncology

2.1.9. Apitegromab - Orphan - EMEA-002951-PIP02-21

Scholar Rock, Inc.; Treatment of spinal muscular atrophy

Day 120 opinion

Action: For adoption

Other / Neurology

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

Treatment of anaemias due to chronic kidney disorders

Day 120 opinion

Action: For adoption

Uro-nephrology

2.1.11. Zigakibart - Orphan - EMEA-003300-PIP01-22

Chinook Therapeutics, Inc.; Treatment of primary IgA nephropathy

Day 120 opinion

Action: For adoption

Uro-nephrology

2.1.12. MRNA encoding for the linked NTD and RBD domains of the spike glycoprotein of SARS-CoV-2 - EMEA-003426-PIP01-23

Prevention of coronavirus disease 2019 (COVID-19)

Day 120 opinion

Action: For adoption

Vaccines / Infectious Diseases

2.1.13. Ezetimibe / obicetrapib - EMEA-003514-PIP01-23

Treatment of mixed hyperlipidaemia

Day 60 opinion

Action: For adoption

Cardiovascular Diseases

2.1.14. Ezetimibe / obicetrapib - EMEA-003514-PIP02-23

Treatment of elevated cholesterol

Day 60 opinion

Action: For adoption

Cardiovascular Diseases

2.1.15. Ruxolitinib - EMEA-002618-PIP04-23

Treatment of *Prurigo nodularis*

Day 60 opinion

Action: For adoption

Dermatology

2.1.16. EMEA-003503-PIP01-23

Treatment of eosinophilic granulomatosis with polyangiitis (EGPA)

Day 60 opinion

Action: For adoption

Immunology-Rheumatology-Transplantation

2.1.17. Nipocalimab - Orphan - EMEA-002559-PIP09-23

Janssen-Cilag International NV; Prevention of foetal and neonatal alloimmune thrombocytopenia

Day 60 opinion

Action: For adoption

Immunology-Rheumatology-Transplantation

2.1.18. Cladribine - EMEA-000383-PIP04-23

Treatment of myasthenia gravis

Day 60 opinion

Action: For adoption

Neurology

2.1.19. Tifcemalimab - EMEA-003512-PIP01-23

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

Day 60 opinion

Action: For adoption

Oncology

2.1.20. Tinengotinib - EMEA-003504-PIP01-23

Treatment of cholangiocarcinoma

Day 60 opinion

Action: For adoption

Oncology

2.1.21. Faricimab - EMEA-002817-PIP05-23

Treatment of choroidal neovascularisation secondary to pathologic myopia

Day 60 opinion

Action: For adoption

Ophthalmology

2.2. Opinions on Compliance Check

2.2.1. Belimumab - EMEA-C-000520-PIP02-13-M04

Glaxo Group Limited; Treatment of systemic lupus erythematosus

Day 60 opinion

Action: For adoption

Immunology-Rheumatology-Transplantation

2.2.2. Bosutinib - EMEA-C-000727-PIP01-09-M07

Pfizer Europe MA EEIG; Treatment of chronic myeloid leukaemia

Day 60 opinion

Action: For adoption

Oncology

2.2.3. Isatuximab - EMEA-C-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

2.2.4. Vorasidenib (as hemicitrate, hemihydrate salt) - EMEA-C-002932-PIP02-21

Les Laboratoires Servier; Treatment of low grade glioma

Day 30 opinion

Action: For adoption

Oncology

2.2.5. Remdesivir - EMEA-C-002826-PIP01-20-M04

Gilead Sciences International Ltd.; Treatment of coronavirus disease 2019 (COVID-19)

Day 30 opinion

Action: For adoption

Infectious Diseases

2.2.6. *Dermatophagoides pteronyssinus* / *Dermatophagoides farinae* - EMEA-C-001258-PIP01-11-M08

ALK-Abelló A/S; Treatment of asthma

Day 30 opinion

Action: For adoption

Pneumology - Allergology

2.3. Opinions on Modification of an Agreed Paediatric Investigation Plan

2.3.1. Etripamil - EMEA-002303-PIP01-17-M04

Milestone Pharmaceuticals, Inc.; Treatment of supraventricular arrhythmia

Day 60 opinion

Action: For adoption

Cardiovascular Diseases

2.3.2. Deucravacitinib - EMEA-002350-PIP01-18-M03

Bristol-Myers Squibb Pharma EEIG; Treatment of psoriasis

Day 60 opinion

Action: For adoption

Dermatology

2.3.3. Tralokinumab - EMEA-001900-PIP02-17-M08

LEO Pharma A/S; Treatment of atopic dermatitis

Day 60 opinion

Action: For adoption

Dermatology

2.3.4. Regadenoson - EMEA-000410-PIP01-08-M06

GE Healthcare AS; Diagnosis of myocardial perfusion disturbances

Day 60 opinion

Action: For adoption

Diagnostic / Cardiovascular Diseases

2.3.5. Pariglasgene brecaparvovec (DTX401) - Orphan - EMEA-002734-PIP01-19-M01

Ultragenyx Germany GmbH; Treatment of glycogen storage disease type Ia

Day 60 opinion

Action: For adoption

Endocrinology-Gynaecology-Fertility-Metabolism

2.3.6. Tolvaptan - EMEA-001231-PIP02-13-M11

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

Day 60 opinion

Action: For adoption

Endocrinology-Gynaecology-Fertility-Metabolism / Uro-nephrology

2.3.7. Crizanlizumab - Orphan - EMEA-002141-PIP01-17-M04

Novartis Europharm Limited; Treatment of sickle cell disease

Day 60 opinion

Action: For adoption

Haematology-Hemostaseology

2.3.8. Mozafancogene autotemcel - Orphan - EMEA-002578-PIP01-19-M01

Rocket Pharmaceuticals, Inc; Treatment of Fanconi anaemia subtype A

Day 60 opinion

Action: For adoption

Haematology-Hemostaseology

2.3.9. Apremilast - EMEA-000715-PIP02-11-M07

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

Day 60 opinion

Action: For adoption

Immunology-Rheumatology-Transplantation

Note: Withdrawal request received on 28 November 2023

2.3.10. Tocilizumab - EMEA-000309-PIP07-21-M01

Roche Registration GmbH; Treatment of coronavirus disease 2019 (COVID-19)

Day 60 opinion

Action: For adoption

Immunology-Rheumatology-Transplantation

2.3.11. Upadacitinib - EMEA-001741-PIP01-14-M07

AbbVie Ltd; Treatment of chronic idiopathic arthritis

Day 60 opinion

Action: For adoption

Immunology-Rheumatology-Transplantation

2.3.12. Ceftobiprole medocaril sodium - EMEA-000205-PIP02-11-M06

Basilea Pharmaceutica Deutschland GmbH; Treatment of pneumonia

Day 60 opinion

Action: For adoption

Infectious Diseases

2.3.13. Ivosidenib - Orphan - EMEA-002247-PIP03-17-M01

Les Laboratoires Servier; Treatment of acute myeloid leukaemia

Day 60 opinion

Action: For adoption

Oncology

2.3.14. Odronextamab - Orphan - EMEA-003149-PIP01-21-M02

Regeneron Ireland DAC; Treatment of mature B cell malignancies

Day 60 opinion

Action: For adoption

Oncology

2.3.15. Bupivacaine - EMEA-000877-PIP03-17-M05

Pacira Ireland Ltd; Postsurgical analgesia

Day 60 opinion

Action: For adoption

Pain

2.3.16. Pegcetacoplan - Orphan - EMEA-002600-PIP03-21-M01

Apellis Ireland Limited; Treatment of glomerulonephritis and nephrotic syndrome

Day 60 opinion

Action: For adoption

Uro-nephrology

2.3.17. Dengue tetravalent vaccine (live, attenuated) - EMEA-001888-PIP01-15-M02

Takeda Vaccines, Inc.; Prevention of dengue fever

Day 60 opinion

Action: For adoption

Vaccines

2.3.18. Chikungunya virus, strain CHIKV LR2006-OPY1, live attenuated - EMEA-002873-PIP01-20-M01

Valneva Austria GmbH; Prevention of chikungunya disease

Day 30 opinion

Action: For information

Vaccines

The PDCO adopted the opinion by written procedure on 23 November 2023

2.3.19. 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) - EMEA-003025-PIP03-23-M01

ExCellThera; Treatment in allogeneic haematopoietic stem cell transplantation in patients with haematological malignancies

Day 30 opinion

Action: For opinion

Oncology; Haematology-Hemostaseology; Immunology-Rheumatology-Transplantation

2.3.20. (R)-1-(1-acryloylpiperidin-3-yl)-4-amino-3-(4-phenoxyphenyl)-1H-imidazo[4,5-c]pyridin-2(3H)-one - EMEA-002566-PIP01-19-M01

Sanofi Winthrop Industrie; Treatment of multiple sclerosis

Day 30 opinion

Action: For adoption

Neurology

2.3.21. Gadopiclenol - EMEA-001949-PIP01-16-M06

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

Day 30 opinion

Action: For adoption

Diagnostic

2.3.22. Gadopiclenol - EMEA-001949-PIP02-18-M04

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

Day 30 opinion

Action: For adoption

Diagnostic

2.4. Opinions on Re-examinations

2.4.1. Amlitelimab - EMEA-003233-PIP01-22

Sanofi Winthrop Industrie; Treatment of atopic dermatitis

Day 30 opinion

Action: For adoption. Oral explanation to be held on Wednesday 13 December 2023 at 14:00

Dermatology

2.4.2. Cedazuridine / decitabine - EMEA-003071-PIP02-23

Otsuka Pharmaceutical Netherlands B.V.; Treatment of myelodysplastic syndromes

Day 30 opinion

Action: For adoption

Oncology / Haematology-Hemostaseology

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. Botaretigene sparoparvovec - EMEA-C1-002827-PIP01-20-M02

Janssen-Cilag International N.V.; Treatment of retinitis pigmentosa

Day 30 letter

Action: For information

Ophthalmology

2.7.2. Chikungunya virus, strain CHIKV LR2006-OPY1, live attenuated - EMEA-C1-002873-PIP01-20

Valneva Austria GmbH; Prevention of chikungunya disease

Day 30 letter

Action: For information

Vaccines

Note: Withdrawal received on 23 November 2023

2.7.3. Remibrutinib - EMEA-C2-002582-PIP01-19-M03

Novartis Europharm Limited; Treatment of chronic spontaneous urticaria

Day 30 letter

Action: For information

Dermatology

2.7.4. Iptacopan - EMEA-C2-002705-PIP03-20

Novartis Europharm Limited; Treatment of paroxysmal nocturnal haemoglobinuria

Day 30 letter

Action: For information

Other / Haematology-Hemostaseology

3. Discussion of applications

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

3.1. Discussions on Products D90-D60-D30

3.1.1. Upadacitinib - EMEA-001741-PIP10-23

Treatment of alopecia areata

Day 90 discussion

Action: For discussion

Dermatology

3.1.2. EMEA-003299-PIP02-22

Treatment of obesity

Day 90 discussion

Action: For discussion

Endocrinology-Gynaecology-Fertility-Metabolism

3.1.3. Sepiapterin - Orphan - EMEA-003027-PIP02-23

PTC Therapeutics International; Treatment of hyperphenylalaninaemia

Day 90 discussion

Action: For discussion

Endocrinology-Gynaecology-Fertility-Metabolism

3.1.4. Synthetic double-stranded siRNA oligonucleotide directed against apolipoprotein C-III mRNA and covalently linked to a ligand containing three N-acetylgalactosamine residues - Orphan - EMEA-003420-PIP01-23

Arrowhead Pharmaceuticals, Inc.; Treatment of familial chylomicronemia syndrome (FCS)

Day 90 discussion

Action: For discussion

Endocrinology-Gynaecology-Fertility-Metabolism

3.1.5. Dupilumab - EMEA-001501-PIP12-23

Treatment of eosinophilic gastritis / gastroenteritis

Day 90 discussion

Action: For discussion

Gastroenterology-Hepatology

3.1.6. Ianalumab - EMEA-002338-PIP04-23

Treatment of autoimmune haemolytic anaemia

Day 90 discussion

Action: For discussion

Haematology-Hemostaseology

3.1.7. Belumosudil - Orphan - EMEA-003425-PIP01-23

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

Day 90 discussion

Action: For discussion

Immunology-Rheumatology-Transplantation

3.1.8. Blinatumomab - Orphan - EMEA-000574-PIP03-23

Amgen Europe B.V.; Treatment of B-lymphoblastic leukaemia/lymphoma

Day 90 discussion

Action: For discussion

Oncology

3.1.9. Clobetasol - EMEA-003458-PIP01-23

Treatment of ocular infections, inflammations and associated manifestations

Day 90 discussion

Action: For discussion

Ophthalmology

3.1.10. Tinlarebant - Orphan - EMEA-003225-PIP01-22

Belite Bio, Inc; Treatment of Stargardt disease

Day 90 discussion

Action: For discussion

Ophthalmology

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

Sanofi B.V.; Treatment of Fabry disease

Day 60 discussion

Action: For discussion

Endocrinology-Gynaecology-Fertility-Metabolism

3.1.12. EMEA-003513-PIP01-23

Treatment of coeliac disease

Day 60 discussion

Action: For discussion

Gastroenterology-Hepatology

3.1.13. Mezagitamab - EMEA-003502-PIP01-23

Treatment of immune thrombocytopenia (ITP)

Day 60 discussion

Action: For discussion

Haematology-Hemostaseology

3.1.14. Recombinant humanised IgG1, kappa light chain, long-acting monoclonal antibody - EMEA-003510-PIP01-23

Prevention of hereditary angioedema attacks

Day 60 discussion

Action: For discussion

Haematology-Hemostaseology

3.1.15. Zunsemetinib - EMEA-003511-PIP01-23

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

Day 60 discussion

Action: For discussion

Immunology-Rheumatology-Transplantation

3.1.16. Humanised IgG1K monoclonal antibody against interferon beta - Orphan - EMEA-003089-PIP02-23

Pfizer Europe MA EEIG; Treatment of idiopathic inflammatory myopathy (ICD11 4A41)

Day 60 discussion

Action: For discussion

Immunology-Rheumatology-Transplantation / Dermatology

3.1.17. Contezolid - EMEA-003508-PIP01-23

Acute bacterial skin and skin structure infection (ABSSSI) / Moderate to severe diabetic foot infection (DFI) without concomitant osteomyelitis

Day 60 discussion

Action: For discussion

Infectious Diseases

3.1.18. Contezolid acefosamil - EMEA-003509-PIP01-23

Acute bacterial skin and skin structure infection (ABSSSI) / Moderate to severe diabetic foot infection (DFI) without concomitant osteomyelitis

Day 60 discussion

Action: For discussion

Infectious Diseases

3.1.19. Sonrotoclax - EMEA-003489-PIP02-23

Treatment of malignant solid tumours

Day 60 discussion

Action: For discussion

Oncology

3.1.20. Multivalent, recombinant, N-terminal surface protein vaccine, containing the alpha-like proteins Rib, AlpC, Alp1, Alp 2/3 antigens of *Streptococcus agalactiae* - EMEA-003505-PIP01-23

Prevention of group B streptococcal invasive disease in infants through maternal immunisation

Day 60 discussion

Action: For discussion

Vaccines

3.1.21. Doruxapapogenum ralaplasmidum (pGX3024) - Orphan - EMEA-003506-PIP01-23

Inovio, Inc.; Treatment of papilloma viral infections

Day 60 discussion

Action: For discussion

Vaccines / Infectious Diseases

3.1.22. Human, recombinant, non-fucosylated IgG1k monoclonal antibody targeting OX-40 receptor on activated T cells (rocatinlimab) - EMEA-002886-PIP02-23

Treatment of *Prurigo nodularis*

Day 30 discussion

Action: For discussion

Dermatology

3.1.23. Pegtibatinase - Orphan - EMEA-003518-PIP01-23

Travere Therapeutics Ireland Limited; Treatment of classical homocystinuria / Classical homocystinuria due to CBS-deficiency

Day 30 discussion

Action: For discussion

Endocrinology-Gynaecology-Fertility-Metabolism

3.1.24. Nomacopan - Orphan - EMEA-003517-PIP01-23

Akari Malta Ltd; Treatment in haematopoietic stem cell transplantation

Day 30 discussion

Action: For discussion

Haematology-Hemostaseology

3.1.25. Ensitrelvir - EMEA-003192-PIP02-23

Treatment of coronavirus disease 2019 (COVID-19)

Day 30 discussion

Action: For discussion

Infectious Diseases

3.1.26. Lenacapavir - EMEA-002740-PIP02-23

Prevention of human immunodeficiency virus (HIV-1) infection

Day 30 discussion

Action: For discussion

Infectious Diseases

3.1.27. EMEA-003515-PIP01-23

Treatment of Parkinson's disease

Day 30 discussion

Action: For discussion

Neurology

3.1.28. Iptacopan - EMEA-002705-PIP06-23

Treatment of generalised myasthenia gravis (gMG)

Day 30 discussion

Action: For discussion

Neurology

3.1.29. IgG-like T cell engager binding to DLL3 and CD3 - EMEA-003516-PIP01-23

Treatment of neuroendocrine carcinoma / Treatment of small cell lung carcinoma

Day 30 discussion

Action: For discussion

Oncology

3.1.30. Interferon gamma / Tumour necrosis factor-alpha / Granulocyte colony-stimulating factor / Interleukin-1 beta, human / Interleukin-2 - EMEA-003523-PIP01-23

Treatment of squamous cell carcinoma of the head and neck (SCCHN)

Day 30 discussion

Action: For discussion

Oncology

3.1.31. Isatuximab - EMEA-002205-PIP02-23

Treatment of malignant neoplasms of the haematopoietic and lymphoid tissue

Day 30 discussion

Action: For discussion

Oncology

3.1.32. Volrustomig - EMEA-003423-PIP02-23

Treatment of head and neck squamous cell carcinoma

Day 30 discussion

Action: For discussion

Oncology

3.1.33. Zanzalintinib - EMEA-003522-PIP01-23

Treatment of colorectal cancer / Treatment of renal cell carcinoma / Treatment of head and neck squamous cell carcinoma

Day 30 discussion

Action: For discussion

Oncology

3.1.34. Zenocutuzumab - EMEA-003519-PIP01-23

Treatment of pancreatic cancer / Treatment of lung cancer

Day 30 discussion

Action: For discussion

Oncology

3.1.35. EMEA-003520-PIP01-23

Treatment of dry eye disease

Day 30 discussion

Action: For discussion

Ophthalmology

3.1.36. 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 30 discussion

Action: For discussion

Other

3.1.37. Mirdametinib - Orphan - EMEA-003525-PIP01-23

Springworks Therapeutics Ireland Limited; Treatment of neurofibromatosis type 1 - plexiform neurofibroma

Day 30 discussion

Action: For discussion

Other

3.1.38. Vosoritide - EMEA-002033-PIP02-23

Treatment of hypochondroplasia

Day 30 discussion

Action: For discussion

Other

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

Prevention of herpes zoster

Day 30 discussion

Action: For discussion

Vaccines

3.1.40. Single-stranded 5' capped mRNA encoding the HAs of the influenza virus and the N-terminal domain (NTD) and receptor binding domain (RBD) of the SARS-CoV-2 spike glycoprotein - EMEA-003521-PIP01-23

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

Day 30 discussion

Action: For discussion

Vaccines

3.2. Discussions on Compliance Check

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

3.2.1. Pegfilgrastim - EMEA-C-002671-PIP02-20

Accord Healthcare S.L.U.; Prevention of chemotherapy-induced febrile neutropenia

Day 30 discussion

Action: For discussion

Haematology-Hemostaseology

3.2.2. Casirivimab - EMEA-C-002964-PIP01-21-M02

Roche Registration GmbH; Prevention of coronavirus disease 2019 (COVID-19)

Day 30 discussion

Action: For discussion

Infectious Diseases

3.2.3. Imdevimab - EMEA-C-002965-PIP01-21-M02

Roche Registration GmbH; Prevention of coronavirus disease 2019 (COVID-19)

Day 30 discussion

Action: For discussion

Infectious Diseases

3.2.4. Rilpivirine - EMEA-C1-000317-PIP02-18-M01

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

Day 30 discussion

Action: For discussion

Infectious Diseases

3.2.5. Tozinameran, tozinameran / famtozinameran - EMEA-C1-002861-PIP02-20-M06

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

Day 30 discussion

Action: For discussion

Infectious Diseases

3.2.6. Autologous tumour-infiltrating lymphocytes (LN-144/LN-145) - EMEA-C2-002776-PIP01-20-M02

Iovance Biotherapeutics, Inc.; Treatment of all conditions included in the category of malignant neoplasms (except haematopoietic and lymphoid tissue neoplasms)

Day 30 discussion

Action: For discussion

Oncology

3.2.7. Talimogene laherparepvec - EMEA-C-001251-PIP01-11-M06

Amgen Europe B.V.; Treatment of melanoma

Day 30 discussion

Action: For discussion

Oncology

3.3. Discussions on Modification of an Agreed Paediatric Investigation Plan

3.3.1. Difelikefalin - EMEA-002565-PIP02-19-M01

Vifor Fresenius Medical Care Renal Pharma France; Treatment of chronic kidney disease associated pruritus

Day 30 discussion

Action: For discussion

Dermatology

3.3.2. Azilsartan medoxomil - EMEA-000237-PIP01-08-M12

Takeda Development Centre Europe Ltd; Treatment of hypertension

Day 30 discussion

Action: For discussion

Cardiovascular Diseases

3.3.3. Mepolizumab - EMEA-000069-PIP01-07-M08

GSK Trading Services Limited; Treatment of hypereosinophilic syndrome

Day 30 discussion

Action: For discussion

Haematology-Hemostaseology

3.3.4. Recombinant human A disintegrin and metalloprotease with thrombospondin type-1 motifs 13 (rADAMTS13) - Orphan - EMEA-001160-PIP01-11-M04

Takeda Pharmaceuticals International AG; Treatment of thrombotic thrombocytopenic purpura

Day 30 discussion

Action: For discussion

Haematology-Hemostaseology

3.3.5. Oritavancin (diphosphate) - EMEA-001270-PIP01-12-M07

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

Day 30 discussion

Action: For discussion

Infectious Diseases

3.3.6. Acetyl-L-leucine ((s)-(acetylamino)-4-methylpentanoic acid) - Orphan - EMEA-002796-PIP01-20-M02

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

Day 30 discussion

Action: For discussion

Neurology

3.3.7. Fordadistrogene movaparvovec - Orphan - EMEA-002741-PIP01-20-M02

Pfizer Europe MA EEIG; Treatment of Duchenne muscular dystrophy

Day 30 discussion

Action: For discussion

Neurology

3.3.8. Givinostat - Orphan - EMEA-000551-PIP04-21-M03

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

Day 30 discussion

Action: For discussion

Neurology

3.3.9. Abemaciclib - EMEA-002342-PIP01-18-M04

Eli Lilly and Company Limited; Treatment of Ewing's sarcoma

Day 30 discussion

Action: For discussion

Oncology

3.3.10. Abemaciclib - EMEA-002342-PIP02-18-M03

Eli Lilly and Company Limited; Treatment of glioma

Day 30 discussion

Action: For discussion

Oncology

3.3.11. Midostaurin - Orphan - EMEA-000780-PIP01-09-M07

Novartis Europharm Limited; Treatment of acute myeloid leukaemia / Treatment of mast cell leukaemia / Treatment of malignant mastocytosis

Day 30 discussion

Action: For discussion

Oncology

3.3.12. Atropine sulfate - EMEA-002744-PIP01-19-M01

Nevakar Inc.; Treatment of myopia

Day 30 discussion

Action: For discussion

Ophthalmology

3.3.13. Molgramostim - Orphan - EMEA-002282-PIP01-17-M02

Savara Aps; Treatment of pulmonary alveolar proteinosis

Day 30 discussion

Action: For discussion

Pneumology - Allergology

3.3.14. Nedosiran - Orphan - EMEA-002493-PIP01-18-M06

Novo Nordisk A/S; Treatment of primary hyperoxaluria

Day 30 discussion

Action: For discussion

Uro-nephrology

3.3.15. Purified rabies virus, WISTAR PM/WI 38-1503-3M strain (inactivated) - EMEA-002234-PIP01-17-M02

Sanofi Pasteur; Prevention of rabies viral infection

Day 30 discussion

Action: For discussion

Vaccines

3.3.16. Recombinant SARS-CoV-2 spike protein - EMEA-002915-PIP01-20-M03

Sanofi Pasteur; Prevention of coronavirus disease 2019 (COVID-19)

Day 30 discussion

Action: For discussion

Vaccines

3.3.17. Chikungunya virus virus-like particle vaccine / aluminum hydroxide - EMEA-002656-PIP01-19-M01

Bavarian Nordic A/S; Prevention of chikungunya disease

Day 30 discussion

Action: For discussion

Vaccines / Infectious Diseases

3.3.18. Sodium zirconium cyclosilicate - EMEA-001539-PIP01-13-M06

AstraZeneca AB; Treatment of hyperkalaemia

Day 30 discussion

Action: For discussion

Endocrinology-Gynaecology-Fertility-Metabolism

3.3.19. Eluxadoline - EMEA-001579-PIP01-13-M06

AbbVie Limited; Treatment of diarrhoea-predominant irritable bowel syndrome

Day 30 discussion

Action: For discussion

Gastroenterology-Hepatology

3.3.20. Upadacitinib - EMEA-001741-PIP04-17-M05

AbbVie Ltd; Treatment of atopic dermatitis

Day 30 discussion

Action: For discussion

Immunology-Rheumatology-Transplantation; Dermatology

3.3.21. Dinutuximab beta - EMEA-001314-PIP01-12-M02

Recordati Netherlands B.V.; Treatment of neuroblastoma

Day 30 discussion

Action: For discussion

Oncology

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 3 January 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. Alvelestat - EMEA-10-2023

Mereo BioPharma Ireland Limited; All classes of medicinal products for 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) / Treatment of severe AATD-associated lung disease (AATD-LD), specifically emphysema

Action: For adoption

6.1.2. Mitiperstat - EMEA-12-2023

AstraZeneca AB; All classes of medicinal products for 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) / Treatment of COPD with a history of exacerbations

Action: For adoption

6.1.3. Adeno-associated virus serotype R100 containing VEGF-C and aflibercept transgene - EMEA-13-2023

4D Molecular Therapeutics, Inc.; All classes of medicinal products for treatment of age-related macular degeneration and diabetic macular oedema / Treatment of age-related macular degeneration (AMD) and diabetic macular oedema (DME)

Action: For adoption

6.1.4. EMEA-14-2023

Janssen-Cilag International NV; All classes of medicinal products for treatment of Alzheimer’s disease / Treatment of preclinical Alzheimer’s disease

Action: For adoption

7. Discussion on the inclusion of 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) - 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

9.2.2. Reflection paper on primary biliary cholangitis (PBC) / primary sclerosing cholangitis (PSC)

PDCO member: Peter Szitanyi

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)

Action: For information

9.3.4. Upcoming Innovation Task Force (ITF) meetings

Action: For information

9.3.5. Methodology Working Party (MWP) - Presentation of reflection paper on use of real-world data to generate real-world evidence in non-interventional studies

Expert: Stine Hasling Mogensen

Action: For discussion

9.4. Cooperation within the EU regulatory network

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

No item

9.5. Cooperation with International Regulators

9.5.1. Paediatric Cluster Teleconference

Action: For information

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

No item

9.7. PDCO work plan

9.7.1. Workplan for 2024

PDCO Chair: Brian Aylward

Action: For adoption

9.8. Planning and reporting

9.8.1. EMA Business Pipeline activity and Horizon scanning

Q4/2023 Update of the Business Pipeline report for the human scientific committees

Action: For information

10. Any other business

10.1. Real World Evidence update, including DARWIN EU®

Action: For information

10.2. Research project in paediatric oncology - from PIP to MA - lessons learnt

Action: For information

10.3. First-line tuberculosis products for paediatric patients - EC initiative

PDCO Chair: Brian Aylward

Action: For information

11. Breakout sessions

11.1. Internal PDCO Operations

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

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

11.5. 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/