



EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

12 October 2021
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Human Medicines Division

Paediatric Committee (PDCO)

Draft Agenda for the meeting on 12-15 October 2021

Chair: Koenraad Norga – Vice-Chair: Sabine Scherer

12 October 2021, 14:00- 19:00, virtual meeting

13 October 2021, 08:30- 19:00, virtual meeting

14 October 2021, 08:30- 19:00, virtual meeting

15 October 2021, 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 12-15 October 2021. See October 2021 PDCO minutes (to be published post November 2021 PDCO meeting).

1.2. Adoption of agenda

PDCO agenda for 12-15 October 2021

1.3. Adoption of the minutes

PDCO minutes for 07-10 September 2021

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. Peptide derivative of glucagon-like-peptide 1 and glucagon with fatty acid side chain - EMEA-002942-PIP01-20

Treatment of non-alcoholic steatohepatitis

Day 120 opinion

Action: For adoption

Gastroenterology-Hepatology

2.1.2. Marzeptacog alfa (activated) - Orphan - EMEA-002270-PIP03-20

Catalyst Biosciences, Inc.; Treatment of haemophilia A

Day 120 opinion

Action: For adoption

Haematology-Hemostaseology

2.1.3. Marzeptacog alfa (activated) - Orphan - EMEA-002270-PIP04-20

Catalyst Biosciences, Inc.; Treatment of haemophilia B

Day 120 opinion

Action: For adoption

Haematology-Hemostaseology

2.1.4. Ravulizumab - EMEA-001943-PIP04-20

Aquaporin-4 antibody-positive neuromyelitis optica spectrum disorder

Day 120 opinion

Action: For adoption

Neurology

2.1.5. Satralizumab - Orphan - EMEA-001625-PIP02-21

Roche Registration GmbH; Treatment of myasthenia gravis

Day 120 opinion

Action: For adoption

Neurology

2.1.6. Lutetium (¹⁷⁷Lu) oxodotreotide - Orphan - EMEA-002950-PIP01-20

Advanced Accelerator Applications; Gastroenteropancreatic neuroendocrine tumours (GEP-NETs)

Day 120 opinion

Action: For adoption

Oncology

2.1.7. Magrolimab - Orphan - EMEA-002819-PIP01-20

Gilead Sciences International Ltd; Treatment of acute myeloid leukaemia / Treatment of myelodysplastic syndromes (including juvenile myelomonocytic leukaemia)

Day 120 opinion

Action: For adoption

Oncology

2.1.8. 2'-O-(2-methoxyethyl) phosphorothioate antisense oligonucleotide targeting CD49d RNA - Orphan - EMEA-002981-PIP01-21

Antisense Therapeutics Limited; Treatment of Duchenne muscular dystrophy

Day 120 opinion

Action: For adoption

Other

2.1.9. Evenamide - EMEA-002519-PIP03-21

Treatment of schizophrenia

Day 120 opinion

Action: For adoption

Psychiatry

2.1.10. *Neisseria meningitidis* serogroup B Protein-based active substance / Recombinant *Neisseria meningitidis* serogroup B protein 1 / Recombinant *Neisseria meningitidis* serogroup B protein 3 / Recombinant *Neisseria meningitidis* serogroup B protein 2 - EMEA-002954-PIP02-21

Prevention of meningococcal disease

Day 120 opinion

Action: For adoption

Vaccines

2.1.11. Amlodipine / ramipril - EMEA-003070-PIP01-21

Treatment of hypertension

Day 60 opinion

Action: For adoption

Cardiovascular Diseases

2.1.12. Furosemide / eplerenone - EMEA-003065-PIP01-21

Treatment of heart failure

Day 60 opinion

Action: For adoption

Cardiovascular Diseases

2.1.13. Verdiperstat - Orphan - EMEA-002708-PIP02-21

Biohaven Pharmaceutical Ireland DAC; Treatment of amyotrophic lateral sclerosis

Day 60 opinion

Action: For adoption

Neurology

2.1.14. Adagrasib - EMEA-003068-PIP01-21

Treatment of non-small cell lung cancer

Day 60 opinion

Action: For adoption

Oncology

2.1.15. Adagrasib - EMEA-003068-PIP02-21

Treatment of colorectal cancer

Day 60 opinion

Action: For adoption

Oncology

2.1.16. Adavosertib - EMEA-003069-PIP01-21

Treatment of malignant endometrial neoplasms / Treatment of malignant pancreatic neoplasms

Day 60 opinion

Action: For adoption

Oncology

2.1.17. Ofranergene obadenovec - Orphan - EMEA-003062-PIP01-21

Vascular Biogenics Ltd. (VBL Therapeutics); Treatment of primary peritoneal cancer / Treatment of ovarian cancer / Treatment of fallopian tube cancer

Day 60 opinion

Action: For adoption

Oncology

2.1.18. Pamrevlumab - EMEA-002979-PIP03-21

Treatment of pancreatic cancer

Day 60 opinion

Action: For adoption

Oncology

2.1.19. [Vibostolimab / pembrolizumab - EMEA-003063-PIP01-21](#)

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

Day 60 opinion

Action: For adoption

Oncology

2.1.20. [Otenaproxesul - EMEA-003061-PIP01-21](#)

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

Day 60 opinion

Action: For adoption

Pain

2.1.21. [Benralizumab - EMEA-001214-PIP08-21](#)

Treatment of non-cystic fibrosis bronchiectasis with an eosinophilic phenotype

Day 60 opinion

Action: For adoption

Pneumology - Allergology

2.1.22. [Bis-\(3-deoxy-3-\(4-\(3-fluorophenyl\)-1H-1,2,3-triazol-1-yl\)-beta-D-galactopyranosyl\) sulfane - Orphan - EMEA-003060-PIP01-21](#)

Galecto Biotech AB; Idiopathic pulmonary fibrosis

Day 60 opinion

Action: For adoption

Pneumology - Allergology

2.1.23. [Depemokimab - EMEA-003051-PIP02-21](#)

Eosinophilic granulomatosis with polyangiitis (EGPA)

Day 60 opinion

Action: For adoption

Pneumology – Allergology

Note: Withdrawal request received on 29 September 2021

2.1.24. Depemokimab - EMEA-003051-PIP03-21

Treatment of hypereosinophilic syndrome (HES)

Day 60 opinion

Action: For adoption

Pneumology – Allergology

Note: Withdrawal request received on 29 September 2021

2.1.25. Human alpha1-proteinase inhibitor (also called Alpha-1 Antitrypsin) - EMEA-001525-PIP02-21

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

Day 60 opinion

Action: For adoption

Pneumology - Allergology

2.1.26. Pamrevlumab - EMEA-002979-PIP02-21

Idiopathic pulmonary fibrosis

Day 60 opinion

Action: For adoption

Pneumology - Allergology

2.1.27. Influenza virus surface antigen (haemagglutinin and neuraminidase) of strain A/H5N1 - EMEA-002869-PIP02-21

Influenza due to identified zoonotic or pandemic influenza virus

Day 60 opinion

Action: For adoption

Vaccines

Note: Withdrawal request received on 28 September 2021

2.1.28. Inactivated poliovirus: type 3 (Saukett strain) / Inactivated poliovirus: type 2 (MEF-1 strain) / Inactivated poliovirus: type 1 (Mahoney strain) / *Bordetella pertussis* antigen: Pertactin / *Bordetella pertussis* antigen: Filamentous Haemagglutinin /

Bordetella pertussis antigen: Pertussis toxoid / Tetanus toxoid / Diphtheria toxoid - EMEA-003066-PIP01-21

Prevention of infectious diseases caused by *Corynebacterium diphtheriae*, *Clostridium tetani*, *Bordetella pertussis*, Poliovirus types 1, 2 and 3

Day 60 opinion

Action: For adoption

Vaccines / Infectious Diseases

2.1.29. RSV F protein - EMEA-003094-PIP01-21

Prevention of lower respiratory tract disease caused by respiratory syncytial virus

Day 60 opinion

Action: For adoption

Vaccines / Infectious Diseases

2.2. Opinions on Compliance Check

2.2.1. Olipudase alfa - EMEA-C1-001600-PIP01-13-M02

Genzyme Europe B.V.; Treatment of Niemann-Pick disease

Day 60 letter

Action: For adoption

Endocrinology-Gynaecology-Fertility-Metabolism

2.2.2. Ceftobiprole medocaril - EMEA-C1-000205-PIP02-11-M04

Basilea Pharmaceutica International Ltd; Treatment of pneumonia

Day 60 letter

Action: For adoption

Infectious Diseases

2.2.3. Oseltamivir - EMEA-C-000365-PIP01-08-M12

Roche Registration GmbH; Treatment and prevention of influenza

Day 60 opinion

Action: For adoption

Infectious Diseases

2.2.4. Zanubrutinib - EMEA-C1-002354-PIP02-18

BeiGene Ireland Ltd; Treatment of mature B-cell neoplasms (excluding lymphoplasmacytic lymphoma)

Day 60 letter

Action: For adoption

Oncology

2.2.5. Dolutegravir (DTG) - EMEA-C-000409-PIP01-08-M06

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

Day 30 opinion

Action: For adoption

Infectious Diseases

2.3. Opinions on Modification of an Agreed Paediatric Investigation Plan

2.3.1. Bilastine - EMEA-000347-PIP02-16-M03

Faes Farma S.A.; Treatment of allergic conjunctivitis

Day 60 opinion

Action: For adoption

2.3.2. 3,4-Dimethoxy-N-methylbenzohydroxamic acid / Deferoxamine mesylate / Alfa-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-PIP03-20-M01

Dr. Franz Köhler Chemie GmbH; Heart transplantation

Day 60 opinion

Action: For adoption

Cardiovascular Diseases

2.3.3. Azilsartan medoxomil - EMEA-000237-PIP01-08-M10

Takeda Development Centre Europe Ltd; Treatment of hypertension

Day 60 opinion

Action: For adoption

Cardiovascular Diseases

2.3.4. Macitentan - Orphan - EMEA-001032-PIP01-10-M04

Janssen-Cilag International NV; Treatment of pulmonary arterial hypertension / Treatment of idiopathic pulmonary fibrosis / Treatment of systemic sclerosis

Day 60 opinion

Action: For adoption. Oral explanation to be held on Wednesday, 13 October at 14:30

Cardiovascular Diseases

2.3.5. Vericiguat - EMEA-001636-PIP01-14-M02

Bayer AG; Treatment of left ventricular failure

Day 60 opinion

Action: For adoption

Cardiovascular Diseases

2.3.6. Glycopyrronium bromide - EMEA-002383-PIP01-18-M01

Dr. August Wolff GmbH & Co. KG - Arzneimittel; Treatment of hyperhidrosis

Day 60 opinion

Action: For adoption

Dermatology

2.3.7. Cotadutide - EMEA-002287-PIP01-17-M03

AstraZeneca AB; Treatment of type 2 diabetes mellitus

Day 60 opinion

Action: For adoption

Endocrinology-Gynaecology-Fertility-Metabolism

2.3.8. Evinacumab - EMEA-002298-PIP01-17-M03

Regeneron Ireland DAC; Treatment of elevated cholesterol

Day 60 opinion

Action: For adoption

Endocrinology-Gynaecology-Fertility-Metabolism

2.3.9. Sodium zirconium cyclosilicate - EMEA-001539-PIP01-13-M05

AstraZeneca AB; Treatment of hyperkalaemia

Day 60 opinion

Action: For adoption

Endocrinology-Gynaecology-Fertility-Metabolism

2.3.10. Naloxegol - EMEA-001146-PIP01-11-M07

Kyowa Kirin Pharmaceutical Development Limited; Treatment of opioid-induced constipation

Day 60 opinion

Action: For adoption

Gastroenterology-Hepatology

2.3.11. Marstacimab - Orphan - EMEA-002285-PIP02-19-M01

Pfizer Europe MAA EEIG; Treatment of congenital haemophilia A / Treatment of congenital haemophilia B

Day 60 opinion

Action: For adoption

Haematology-Hemostaseology

2.3.12. Vonicog alfa - EMEA-001164-PIP01-11-M05

Baxalta Innovations GmbH; Treatment of von Willebrand disease

Day 60 opinion

Action: For adoption

Haematology-Hemostaseology

2.3.13. Upadacitinib - EMEA-001741-PIP01-14-M05

AbbVie Ltd; Treatment of chronic idiopathic arthritis

Day 60 opinion

Action: For adoption

Immunology-Rheumatology-Transplantation

2.3.14. Baloxavir marboxil - EMEA-002440-PIP01-18-M02

Roche Registration GmbH; Prevention of influenza / Treatment of influenza

Day 60 opinion

Action: For adoption

Infectious Diseases

2.3.15. Ceftobiprole medocaril sodium - EMEA-000205-PIP02-11-M05

Basilea Pharmaceutica International Ltd.; Treatment of pneumonia

Day 60 opinion

Action: For adoption

Infectious Diseases

2.3.16. Dalbavancin - EMEA-000016-PIP01-07-M08

Allergan Pharmaceuticals International Limited; Treatment of acute bacterial skin and skin structure infections (ABSSSI)

Day 60 opinion

Action: For adoption

Infectious Diseases

2.3.17. Isavuconazonium (sulfate) - Orphan - EMEA-001301-PIP02-12-M04

Basilea Pharmaceutica International Ltd.; Treatment of invasive aspergillosis / Treatment of mucormycosis

Day 60 opinion

Action: For adoption

Infectious Diseases

2.3.18. Oritavancin (diphosphate) - EMEA-001270-PIP01-12-M04

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

Day 60 opinion

Action: For adoption

Infectious Diseases

2.3.19. Pretomanid - Orphan - EMEA-002115-PIP01-17-M04

Global Alliance for TB Drug Development; Treatment of multi-drug-resistant tuberculosis

Day 60 opinion

Action: For adoption

Infectious Diseases

2.3.20. Rilpivirine (RPV) / dolutegravir (DTG) - EMEA-001750-PIP01-15-M05

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

Day 60 opinion

Action: For adoption

Infectious Diseases

2.3.21. Sotrovimab - EMEA-002899-PIP01-20-M01

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

Day 60 opinion

Action: For adoption

Infectious Diseases

2.3.22. Tecovirimat monohydrate - Orphan - EMEA-001205-PIP02-19-M01

SIGA Technologies, Inc.; Orthopoxvirus disease (smallpox, monkeypox, cowpox, and vaccinia complications)

Day 60 opinion

Action: For adoption

Infectious Diseases

2.3.23. Tenofovir alafenamide / emtricitabine / bicitgravir - EMEA-001766-PIP01-15-M04

Gilead Sciences International Ltd.; Treatment of human immunodeficiency virus [HIV] infection

Day 60 opinion

Action: For adoption

Infectious Diseases

2.3.24. Erenumab - EMEA-001664-PIP02-15-M05

Novartis Europharm Limited; Prevention of migraine headaches

Day 60 opinion

Action: For adoption

Neurology

2.3.25. Perampanel - EMEA-000467-PIP01-08-M15

Eisai Europe Limited; Treatment of treatment-resistant epilepsies

Day 60 opinion

Action: For adoption

Neurology

2.3.26. Soticlestat - EMEA-002572-PIP02-19-M01

Takeda Pharma A/S; Dravet syndrome / Lennox-Gastaut syndrome

Day 60 opinion

Action: For adoption

Neurology

2.3.27. Cabozantinib - Orphan - EMEA-001143-PIP01-11-M04

Ipsen Pharma; Treatment of malignant solid tumours

Day 60 opinion

Action: For adoption

Oncology

Note: Withdrawal request received on 23 September 2021

2.3.28. Cyclophosphamide - EMEA-002644-PIP01-19-M01

Accord Healthcare S.L.U.; Treatment of all malignant neoplasms

Day 60 opinion

Action: For adoption

Oncology

2.3.29. Larotrectinib - EMEA-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 60 opinion

Action: For adoption

Oncology

2.3.30. Regorafenib - EMEA-001178-PIP01-11-M06

Bayer AG; Treatment of all conditions contained in the category of malignant neoplasms (except haematopoietic and lymphoid tissue)

Day 60 opinion

Action: For adoption

Oncology

2.3.31. Ruxolitinib phosphate - EMEA-000901-PIP04-17-M02

Novartis Europharm Limited; Chronic Graft versus Host disease

Day 60 opinion

Action: For adoption

Oncology

2.3.32. Inotuzumab ozogamicin - Orphan - EMEA-001429-PIP01-13-M04

Pfizer Europe MA EEIG; Treatment of acute lymphoblastic leukaemia

Day 60 opinion

Action: For adoption

Oncology / Haematology-Hemostaseology

2.3.33. Vamorolone - Orphan - EMEA-001794-PIP02-16-M04

ReveraGen BioPharma Ltd; Treatment of Duchenne muscular dystrophy

Day 60 opinion

Action: For adoption

Other

2.3.34. Methoxflurane - EMEA-000334-PIP01-08-M10

Medical Developments UK Ltd; Treatment of acute pain

Day 60 opinion

Action: For adoption

Pain

2.3.35. Molgramostim - Orphan - EMEA-002282-PIP01-17-M01

Savara Aps; Treatment of pulmonary alveolar proteinosis

Day 60 opinion

Action: For adoption

Pneumology - Allergology

2.3.36. Tezepelumab - EMEA-001613-PIP01-14-M05

AstraZeneca AB; Treatment of asthma

Day 60 opinion

Action: For adoption

Pneumology – Allergology

Note: Withdrawal request received on 27 September 2021

2.3.37. Lumasiran sodium - Orphan - EMEA-002079-PIP01-16-M02

Alnylam UK Limited; Treatment of primary hyperoxaluria type 1

Day 60 opinion

Action: For adoption

Uro-nephrology

2.3.38. Influenza virus surface antigens (haemagglutinin and neuraminidase) of strain B (Victoria lineage) / Influenza virus surface antigens (haemagglutinin and neuraminidase) of strain B (Yamagata lineage) / Influenza virus surface antigens (haemagglutinin and neuraminidase) of strain A (H3N2) / Influenza virus surface antigens (haemagglutinin and neuraminidase) of strain A (H1N1) - EMEA-002068-PIP01-16-M04

Seqirus Netherlands; Influenza

Day 60 opinion

Action: For adoption

Vaccines

2.3.39. NVX-CoV2373 - EMEA-002941-PIP01-20-M01

Novavax CZ, a.s.; Prevention of coronavirus disease 2019 (COVID-19)

Day 60 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. Vadadustat - EMEA-C1-001944-PIP01-16-M02

Otsuka Pharmaceutical Development & Commercialisation Europe GmbH; Treatment of anaemia due to chronic disorders

Day 30 letter

Action: For information

Haematology-Hemostaseology

2.7.2. Bardoxolone - EMEA-C1-002488-PIP01-18

Reata Pharmaceuticals, Inc.; Treatment of Alport syndrome

Day 30 letter

Action: For information

Uro-nephrology

2.7.3. Abacavir (ABC) / lamivudine (3TC) / dolutegravir (DTG) - EMEA-C1-001219-PIP01-11-M05

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

Day 30 letter

Action: For information

Infectious Diseases

2.7.4. Formoterol fumarate dihydrate / glycopyrronium bromide / beclometasone dipropionate - EMEA-C2-001875-PIP02-18-M03

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

Day 30 letter

Action: For information

Pneumology - Allergology

2.7.5. Recombinant human monoclonal antibody against mannan-binding lectin-associated serine protease-2 (INN: narsoplimab) - EMEA-C1-002479-PIP01-18

Omeros Ireland Limited; Treatment in haematopoietic stem cell transplantation

Day 30 letter

Action: For information

Haematology-Hemostaseology

2.7.6. Casirivimab - EMEA-C1-002964-PIP01-21

Regeneron Ireland DAC; Treatment of coronavirus disease 2019 (COVID-19)

Day 30 letter

Action: For information

Infectious Diseases

2.7.7. Imdevimab - EMEA-C1-002965-PIP01-21

Regeneron Ireland DAC; Prevention of coronavirus disease 2019 (COVID-19)

Day 30 letter

Action: For information

Infectious Diseases

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. Autologous CD34+ hematopoietic stem cells with a CRISPR-edited erythroid enhancer region of the BCL11A gene - Orphan - EMEA-002730-PIP03-21

Vertex Pharmaceuticals (Ireland) Limited; Treatment of severe sickle cell disease

Day 90 discussion

Action: For discussion

Haematology-Hemostaseology

3.1.2. Autologous CD34+ hematopoietic stem cells with a CRISPR-edited erythroid enhancer region of the BCL11A gene - Orphan - EMEA-002730-PIP04-21

Vertex Pharmaceuticals (Ireland) Limited; Treatment of beta-thalassemia intermedia and

major

Day 90 discussion

Action: For discussion

Haematology-Hemostaseology

3.1.3. Leniolisib - Orphan - EMEA-002989-PIP01-21

Pharming Group N.V.; Activated phosphoinositide 3-kinase delta syndrome (APDS)

Day 90 discussion

Action: For discussion

Immunology-Rheumatology-Transplantation

3.1.4. Exebacase - EMEA-002947-PIP01-20

Treatment of *Staphylococcus aureus* blood stream infections (bacteraemia)

Day 90 discussion

Action: For discussion

Infectious Diseases

3.1.5. Tosatoxumab - Orphan - EMEA-002506-PIP03-21

Aridis Pharmaceuticals Inc; Treatment of *Staphylococcus aureus* pneumonia

Day 90 discussion

Action: For discussion

Infectious Diseases / Pneumology - Allergology

3.1.6. 2'-O-(2-methoxyethyl) modified antisense oligonucleotide targeting glial fibrillary acidic protein pre-mRNA - Orphan - EMEA-002822-PIP01-20

Ionis Pharmaceuticals; Alexander disease

Day 90 discussion

Action: For discussion

Neurology

3.1.7. Vatiquinone - Orphan - EMEA-001238-PIP03-21

PTC Therapeutics International; Treatment of Friedreich Ataxia

Day 90 discussion

Action: For discussion

Neurology

3.1.8. EMEA-002635-PIP02-21

Treatment of advanced or metastatic malignancies harbouring ALK, ROS1, or NTRK1-3 alterations

Day 90 discussion

Action: For discussion

Oncology

3.1.9. Humanised KLB / FGFR1c monoclonal antibody - EMEA-003058-PIP01-21

Treatment of non-alcoholic steatohepatitis

Day 60 discussion

Action: For discussion

Endocrinology-Gynaecology-Fertility-Metabolism

3.1.10. Semaglutide / cagrilintide - EMEA-003059-PIP01-21

Treatment of obesity

Day 60 discussion

Action: For discussion

Endocrinology-Gynaecology-Fertility-Metabolism

3.1.11. Tezepelumab - EMEA-001613-PIP03-21

Eosinophilic esophagitis

Day 60 discussion

Action: For discussion

Gastroenterology-Hepatology

3.1.12. Clazakizumab - EMEA-001371-PIP02-21

Treatment of chronic active antibody mediated rejection (AMR) in kidney transplantation

Day 60 discussion

Action: For discussion

Immunology-Rheumatology-Transplantation

3.1.13. Human normal immunoglobulin - EMEA-003076-PIP01-21

Treatment of antibody deficiency

Day 60 discussion

Action: For discussion

Immunology-Rheumatology-Transplantation

3.1.14. Retinol palmitate - Orphan - EMEA-003073-PIP01-21

PROVEPHARM SAS; Prevention of bronchopulmonary dysplasia

Day 60 discussion

Action: For discussion

Neonatology - Paediatric Intensive Care

Note: Withdrawal request received on 1 October 2021

3.1.15. Cannabidiol - EMEA-001964-PIP03-21

Treatment of epilepsy with myoclonic atonic seizures

Day 60 discussion

Action: For discussion

Neurology

3.1.16. Censavudine - EMEA-003075-PIP01-21

Treatment of Aicardi-Goutières syndrome

Day 60 discussion

Action: For discussion

Neurology

3.1.17. Autologous tumour-infiltrating lymphocytes (TILs) isolated from a patient's cancer tissue and expanded ex vivo - EMEA-003072-PIP01-21

Treatment of advanced melanoma

Day 60 discussion

Action: For discussion

Oncology

3.1.18. Cedazuridine - EMEA-003071-PIP01-21

Acute myeloid leukaemia

Day 60 discussion

Action: For discussion

Oncology / Haematology-Hemostaseology

3.1.19. Ilofotase alfa - EMEA-003067-PIP01-21

Treatment of sepsis-associated acute kidney injury

Day 60 discussion

Action: For discussion

Uro-nephrology

3.1.20. Lademirsén - Orphan - EMEA-003064-PIP01-21

Genzyme Europe B.V.; Treatment of Alport syndrome

Day 60 discussion

Action: For discussion

Uro-nephrology

3.1.21. Influenza virus surface antigens (haemagglutinin and neuraminidase) of strain A/H5N1 - EMEA-002869-PIP01-21

Influenza due to identified zoonotic or pandemic influenza virus

Day 60 discussion

Action: For discussion

Vaccines

3.1.22. Whole-cell heat-inactivated bacterial strains of *Escherichia coli* / *Klebsiella pneumoniae* / *Proteus vulgaris* / *Enterococcus faecalis* - EMEA-003026-PIP02-21

Prevention of recurrent urinary tract infections (R-UTIs)

Day 60 discussion

Action: For discussion

Vaccines / Infectious Diseases / Uro-nephrology

3.1.23. Efgartigimod alfa - EMEA-002597-PIP07-21

Treatment of pemphigus

Day 30 discussion

Action: For discussion

Dermatology

3.1.24. Venglustat - Orphan - EMEA-001716-PIP06-21

Genzyme Europe B.V.; Treatment of Fabry disease

Day 30 discussion

Action: For discussion

Endocrinology-Gynaecology-Fertility-Metabolism

3.1.25. Resmetirom - EMEA-003087-PIP01-21

Non-alcoholic steatohepatitis (NASH)

Day 30 discussion

Action: For discussion

Gastroenterology-Hepatology

3.1.26. Ritlecitinib - EMEA-002451-PIP02-21

Treatment of ulcerative colitis

Day 30 discussion

Action: For discussion

Gastroenterology-Hepatology

3.1.27. EMEA-003090-PIP01-21

Hereditary angioedema

Day 30 discussion

Action: For discussion

Haematology-Hemostaseology

3.1.28. Deucravacitinib - EMEA-002350-PIP04-21

Treatment of ulcerative colitis (UC)

Day 30 discussion

Action: For discussion

Immunology-Rheumatology-Transplantation

3.1.29. Secukinumab - EMEA-000380-PIP08-21

Treatment of giant cell arteritis / Giant cell arteritis

Day 30 discussion

Action: For discussion

Immunology-Rheumatology-Transplantation

3.1.30. [Humanised IgG1K monoclonal antibody against interferon beta - Orphan - EMEA-003089-PIP01-21](#)

Pfizer Europe MA EEIG; Treatment of dermatomyositis

Day 30 discussion

Action: For discussion

Immunology-Rheumatology-Transplantation / Dermatology

3.1.31. [EMEA-003081-PIP01-21](#)

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

Day 30 discussion

Action: For discussion

Infectious Diseases

3.1.32. [Bepirovirsen - EMEA-003082-PIP01-21](#)

Treatment of chronic hepatitis B infection

Day 30 discussion

Action: For discussion

Infectious Diseases

3.1.33. [Emvododstat - EMEA-003088-PIP01-21](#)

Treatment of coronavirus disease 2019 (COVID-19)

Day 30 discussion

Action: For discussion

Infectious Diseases

3.1.34. [Lonafarnib - Orphan - EMEA-002516-PIP02-21](#)

EigerBio Europe Limited; Hepatitis D virus infection

Day 30 discussion

Action: For discussion

Infectious Diseases

[3.1.35. Molnupiravir - EMEA-002940-PIP02-21](#)

Prevention of coronavirus disease 2019 (COVID-19)

Day 30 discussion

Action: For discussion

Infectious Diseases

[3.1.36. Plitidepsin - Orphan - EMEA-000095-PIP02-21](#)

Pharma Mar, S.A.; Treatment of coronavirus disease 2019 (COVID-19)

Day 30 discussion

Action: For discussion

Infectious Diseases

[3.1.37. Phenylephrine / acetylcysteine / paracetamol - EMEA-003091-PIP01-21](#)

Treatment of upper respiratory tract infections / Treatment of cold and flu-like symptoms with or without fever, mild or moderate pain, nasal congestion and thick mucus secretion

Day 30 discussion

Action: For discussion

Infectious Diseases / Oto-rhino-laryngology

[3.1.38. Ex vivo fused normal allogeneic human myoblast \(MBN\) with autologous human myoblast derived from Duchenne muscular dystrophy affected donor \(MBDMD\) - Orphan - EMEA-003078-PIP01-21](#)

Dystrogen Therapeutics S.A.; Treatment of Duchenne muscular dystrophy

Day 30 discussion

Action: For discussion

Neurology

[3.1.39. Troriluzole - EMEA-003084-PIP01-21](#)

Treatment of hereditary spinocerebellar ataxia

Day 30 discussion

Action: For discussion

Neurology

3.1.40. Derivative of pyrazolo [1,5-a] pyrimidine - EMEA-003086-PIP01-21

Treatment of solid tumours

Day 30 discussion

Action: For discussion

Oncology

3.1.41. CD30-directed genetically modified autologous T cells (CD30.CAR-T) - EMEA-003092-PIP01-21

Treatment of Hodgkin lymphoma

Day 30 discussion

Action: For discussion

Oncology

3.1.42. Humanised IgG2k Fc-modified bispecific monoclonal antibody against CD3 and BCMA - Orphan - EMEA-003083-PIP01-21

Pfizer Europe MA EEIG; Treatment of multiple myeloma

Day 30 discussion

Action: For discussion

Oncology

3.1.43. Milademetan tosilate - Orphan - EMEA-003093-PIP01-21

Rain Therapeutics, Inc.; Dedifferentiated liposarcoma / Liposarcomas malignant

Day 30 discussion

Action: For discussion

Oncology

3.1.44. Benralizumab - EMEA-001214-PIP09-21

Treatment of eosinophilic granulomatosis with polyangiitis (EGPA)

Day 30 discussion

Action: For discussion

Pneumology - Allergology

3.1.45. Humanised IgG2 monoclonal antibody against APRIL - Orphan - EMEA-003085-PIP01-21

Otsuka Pharmaceutical Netherlands B.V.; Treatment of primary IgA nephropathy

Day 30 discussion

Action: For discussion

Uro-nephrology

3.1.46. SARS-CoV-2 virus, beta-propiolactone inactivated - EMEA-003077-PIP01-21

Prevention of 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. Landiolol hydrochloride - EMEA-C1-001150-PIP02-13-M04

Orpha-Devel Handels und Vertriebs GmbH; Treatment of supraventricular arrhythmias

Day 30 discussion

Action: For discussion

Cardiovascular Diseases

3.2.2. Ruxolitinib phosphate - EMEA-C1-002618-PIP02-20

Incyte Biosciences Distribution B.V.; Treatment of vitiligo

Day 30 discussion

Action: For discussion

Dermatology

3.2.3. Cipaglucosidase alfa - EMEA-C3-002447-PIP01-18-M01

Amicus Therapeutics Europe Limited; Treatment of glycogen storage disease Type II (Pompe's disease)

Day 30 discussion

Action: For discussion

Endocrinology-Gynaecology-Fertility-Metabolism

3.2.4. Aflibercept - EMEA-C-000236-PIP05-18

Bayer AG; Retinopathy of prematurity (ROP)

Day 30 discussion

Action: For discussion

Ophthalmology

3.2.5. Fosdenopterin - EMEA-C-001491-PIP01-13-M01

Comharsa Life Sciences Limited; Treatment of molybdenum cofactor deficiency type A

Day 30 discussion

Action: For discussion

Other

3.2.6. Daprodustat - EMEA-C1-001452-PIP01-13-M03

GlaxoSmithKline Trading Services Limited; Treatment of anaemia due to chronic disorders

Day 30 discussion

Action: For discussion

Uro-nephrology

3.3. Discussions on Modification of an Agreed Paediatric Investigation Plan

3.3.1. Nemolizumab - EMEA-001624-PIP01-14-M04

Galderma International S.A.S; Atopic dermatitis

Day 30 discussion

Action: For discussion

Dermatology

3.3.2. Alirocumab - EMEA-001169-PIP01-11-M05

sanofi-aventis recherche & développement; Treatment of elevated cholesterol

Day 30 discussion

Action: For discussion

Endocrinology-Gynaecology-Fertility-Metabolism

3.3.3. Drospirenone / estetrol monohydrate - EMEA-001332-PIP01-12-M05

Estetra SRL; Prevention of pregnancy

Day 30 discussion

Action: For discussion

Endocrinology-Gynaecology-Fertility-Metabolism

3.3.4. Romosozumab - EMEA-001075-PIP04-15-M04

UCB Pharma S.A.; Treatment of osteoporosis

Day 30 discussion

Action: For discussion

Endocrinology-Gynaecology-Fertility-Metabolism

3.3.5. Tirzepatide - EMEA-002360-PIP01-18-M01

Eli Lilly and Company Ltd; Type 2 diabetes mellitus

Day 30 discussion

Action: For discussion

Endocrinology-Gynaecology-Fertility-Metabolism

3.3.6. Vedolizumab - EMEA-000645-PIP01-09-M08

Takeda Pharma A/S; Ulcerative colitis / Crohn's Disease

Day 30 discussion

Action: For discussion

Gastroenterology-Hepatology

3.3.7. Crizanlizumab - Orphan - EMEA-002141-PIP01-17-M03

Novartis Europharm Limited; Treatment of sickle cell disease

Day 30 discussion

Action: For discussion

Haematology-Hemostaseology

3.3.8. Giroctocogene fitelparvovec - Orphan - EMEA-002724-PIP01-19-M02

Pfizer Europe MA EEIG; Treatment of haemophilia A

Day 30 discussion

Action: For discussion

Haematology-Hemostaseology

3.3.9. MK-1654 fully human IgG1 RB-1 YTE anti-RSV F monoclonal antibody - EMEA-002755-PIP01-19-M01

Merck Sharp & Dohme (Europe), Inc.; Prevention of lower respiratory tract infection caused by respiratory syncytial virus

Day 30 discussion

Action: For discussion

Infectious Diseases

3.3.10. Fenfluramine hydrochloride - Orphan - EMEA-001990-PIP01-16-M04

Zogenix International Ltd; Dravet syndrome

Day 30 discussion

Action: For discussion

Neurology

3.3.11. Risdiplam - Orphan - EMEA-002070-PIP01-16-M06

Roche Registration GmbH; Treatment of spinal muscular atrophy

Day 30 discussion

Action: For discussion

Neurology

3.3.12. Avelumab - EMEA-001849-PIP02-15-M04

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

Day 30 discussion

Action: For discussion

Oncology

3.3.13. Eribulin - EMEA-001261-PIP01-11-M07

Eisai GmbH; Soft tissue sarcoma

Day 30 discussion

Action: For discussion

Oncology

3.3.14. Lisocabtagene maraleucel - Orphan - EMEA-001995-PIP01-16-M03

Bristol-Myers Squibb Pharma EEIG; Treatment of B-lymphoblastic leukaemia/lymphoma /
Treatment of mature B-cell neoplasms

Day 30 discussion

Action: For discussion

Oncology

3.3.15. Pembrolizumab - EMEA-001474-PIP02-16-M02

Merck Sharp & Dohme (Europe), Inc.; Treatment of Hodgkin lymphoma

Day 30 discussion

Action: For discussion

Oncology

3.3.16. Alpelisib - Orphan - EMEA-002016-PIP03-19-M01

Novartis Europharm Limited; Treatment of PIK3CA related overgrowth spectrum

Day 30 discussion

Action: For discussion

Other

3.3.17. Human thrombin (component 2) / Human fibrinogen (component 1) - EMEA-001598-PIP01-13-M04

Instituto Grifols, S.A.; Treatment of haemorrhage resulting from a surgical procedure

Day 30 discussion

Action: For discussion

Other

3.3.18. Palovarotene - Orphan - EMEA-001662-PIP01-14-M05

Ipsen Pharma; Treatment of fibrodysplasia ossificans progressiva

Day 30 discussion

Action: For discussion

Other

3.3.19. Synthetic double-stranded siRNA oligonucleotide directed against lactate dehydrogenase A mRNA and containing four modified nucleosides which form a

Dicerna Ireland Limited; Primary hyperoxaluria

Day 30 discussion

Action: For discussion

Uro-nephrology

3.3.20. Tozinameran - EMEA-002861-PIP02-20-M03

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

Day 30 discussion

Action: For discussion

Infectious Diseases

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 October 2021 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. Paltusotine - EMEA-11-2021

The classes of androgen receptor modulator, of 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 treatment of neuroendocrine malignant neoplasms / Treatment of carcinoid syndrome

Action: For discussion

6.1.2. Human plasma Kallikrein inhibitor for intravitreal injection - EMEA-13-2021

All classes of medicinal products for treatment of age-related macular degeneration and diabetic macular oedema / Treatment of diabetic macular oedema

Action: For discussion

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

9.2. 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 Group: D30 Products identified

PDCO member: Karen van Malderen

Action: For information

9.3.2. Formulation Working Group

PDCO member: Brian Aylward

Action: For information

9.4. Cooperation within the EU regulatory network

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

PDCO members: Sabine Scherer, Marek Migdal

Action: For information

9.5. Cooperation with International Regulators

No item

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 2022

PDCO Chair: Koenraad Norga

Action: For discussion

9.8. Planning and reporting

No item

10. Any other business

10.1. COVID-19 update

Action: For information

10.2. Update on resamirigine biloparvovec - EMEA-002571-PIP01-19

Action: For information

10.3. Presentation of the results and conclusions from the extrapolation project

Action: For information

10.4. Registry-based studies guideline – update

Action: For information

10.5. EMA Business Pipeline activity and Horizon scanning

Q3/2021 Update of the Business Pipeline report for the human scientific committees

Action: For information

10.6. Art 45 of the Paediatric Regulation (EC) 1901/2006 – worksharing project update

PDCO member: Siri Wang

Action: For information

10.7.

Action: For discussion

10.8.

Action: For information

11. Breakout sessions

11.1. Neonatology

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

11.2. Paediatric oncology

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

11.3. Vaccines

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

12. Explanatory notes

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

Paediatric investigation plan (PIP) (section 2.1 Opinion on PIPs and section 3.1 Discussions on PIPs)

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

Compliance checks (section 2.2 Opinions on Compliance check, section 3.2 Discussions on Compliance check)

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

Modification of an Agreed Paediatric Investigation Plan (section 2.3 Opinions on Modification of an agreed PIP, section 3.3 Discussions on Modification of an agreed PIP)

The development plan for a medicine can be modified at a later stage as knowledge increases. Modifications can also be made if the applicant encounters such difficulties with the implementation of a PIP, which render it unworkable or no longer appropriate.

In some cases, studies can be deferred until after the studies in adults have been conducted. This ensures that research in children is done only when it is safe and ethical to do so. Even when studies are deferred, the PIP will include details of the paediatric studies and their timelines.

Class waiver (section 6 Discussion on the applicability of class waiver)

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

Annual reports on deferrals (section 8)

If the medicinal product is approved in the EU, annual reports on the deferred measures in the PIP must be submitted to the Agency.

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