09 November 2021
EMA/PDCO/598143/2021
Human Medicines Division

Paediatric Committee (PDCO)

Draft Agenda for the meeting on 9-12 November 2021

Chair: Koenraad Norga – Vice-Chair: Sabine Scherer

9 November 2021, 11:00- 19:00, virtual meeting

10 November 2021, 08:30- 19:00, virtual meeting

11 November 2021, 08:30- 19:00, virtual meeting

12 November 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 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).

Official address Domenico Scarlattilaan 6 ● 1083 HS Amsterdam ● The Netherlands
Address for visits and deliveries Refer to www.ema.europa.eu/how-to-find-us
Send us a question Go to www.ema.europa.eu/contact **Telephone** +31 (0)88 781 6000 An agency of the European Union

© European Medicines Agency, 2021. Reproduction is authorised provided the source is acknowledged.

Table of contents

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 9-12 November 2021. See November 2021 PDCO minutes (to be published post December 2021 PDCO meeting).

1.2. Adoption of agenda

PDCO agenda for 9-12 November 2021

1.3. Adoption of the minutes

PDCO minutes for 12-15 October 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. 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 120 opinion

Action: For adoption

Haematology-Hemostaseology

2.1.2. 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 120 opinion

Action: For adoption

Haematology-Hemostaseology

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

Pharming Group N.V.; Treatment of activated phosphoinositide 3-kinase delta syndrome (APDS)

Day 120 opinion

Action: For adoption. Oral explanation to be held on Wednesday, 10 November at 11:00

Immunology-Rheumatology-Transplantation

2.1.4. Exebacase - EMEA-002947-PIP01-20

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

Day 120 opinion

Action: For adoption

Infectious Diseases

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

Aridis Pharmaceuticals Inc; Treatment of *Staphylococcus aureus* pneumonia

Day 120 opinion

Action: For adoption

Infectious Diseases / Pneumology - Allergology

2.1.6. 2'-O-(2-methoxyethyl) modified antisense oligonucleotide targeting glial fibrillary acidic protein pre-mRNA - Orphan - EMEA-002822-PIP01-20

Ionis Pharmaceuticals; Alexander disease

Day 120 opinion

Action: For adoption

Neurology

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

PTC Therapeutics International; Treatment of Friedreich Ataxia

Day 120 opinion

Action: For adoption

Neurology

2.1.8. EMEA-002635-PIP02-21

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

alterations

Day 120 opinion

Action: For adoption

Oncology

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

Treatment of pemphigus

Day 60 opinion

Action: For adoption

Dermatology

2.1.10. Secukinumab - EMEA-000380-PIP08-21

Giant cell arteritis / Treatment of Giant Cell Arteritis

Day 60 opinion

Action: For adoption

Immunology-Rheumatology-Transplantation

2.1.11. Phenylephrine / acetylcysteine / paracetamol - EMEA-003091-PIP01-21

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

Day 60 opinion

Action: For adoption

Infectious Diseases / Oto-rhino-laryngology

2.1.12. Troriluzole - EMEA-003084-PIP01-21

Treatment of spinocerebellar ataxia

Day 60 opinion

Action: For adoption

Neurology

Note: Withdrawal request received on 29 October 2021

2.1.13. 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 60 opinion

Action: For adoption

Oncology

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

Rain Therapeutics, Inc.; Treatment of liposarcomas

Day 60 opinion

Action: For adoption

Oncology

2.2. Opinions on Compliance Check

2.2.1. Landiolol hydrochloride - EMEA-C1-001150-PIP02-13-M04

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

Day 60 letter

Action: For adoption

Cardiovascular Diseases

Note: Withdrawal request received on 3 November 2021

2.3. Opinions on Modification of an Agreed Paediatric Investigation Plan

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

Galderma International S.A.S; Atopic dermatitis

Day 60 opinion

Action: For adoption

Dermatology

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

sanofi-aventis recherche & développement; Treatment of elevated cholesterol

Day 60 opinion

Action: For adoption

Endocrinology-Gynaecology-Fertility-Metabolism

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

Estetra SRL; Prevention of pregnancy

Day 60 opinion

Action: For adoption

Endocrinology-Gynaecology-Fertility-Metabolism

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

UCB Pharma S.A.; Treatment of osteoporosis

Day 60 opinion

Action: For adoption. Oral explanation to be held on Thursday, 11 November at 11:00

Endocrinology-Gynaecology-Fertility-Metabolism

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

Eli Lilly and Company Ltd; Type 2 diabetes mellitus

Day 60 opinion

Action: For adoption

Endocrinology-Gynaecology-Fertility-Metabolism

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

Takeda Pharma A/S; Treatment of Crohn's disease / Treatment of ulcerative colitis

Day 60 opinion

Action: For adoption

Gastroenterology-Hepatology

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

Novartis Europharm Limited; Treatment of sickle cell disease

Day 60 opinion

Action: For adoption

Haematology-Hemostaseology

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

Pfizer Europe MA EEIG; Treatment of haemophilia A

Day 60 opinion

Action: For adoption

Haematology-Hemostaseology

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

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

Day 60 opinion

Action: For adoption

Infectious Diseases

2.3.10. 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 60 opinion

Action: For adoption

Infectious Diseases

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

Zogenix International Ltd; Dravet syndrome

Day 60 opinion

Action: For adoption

Neurology

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

Roche Registration GmbH; Treatment of spinal muscular atrophy

Day 60 opinion

Action: For adoption

Neurology

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

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

Day 60 opinion

Action: For adoption

Oncology

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

Eisai GmbH; Soft tissue sarcoma

Day 60 opinion

Action: For adoption

Oncology

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

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

Day 60 opinion

Action: For adoption

Oncology

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

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

Day 60 opinion

Action: For adoption

Oncology

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

Novartis Europharm Limited; Treatment of PIK3CA related overgrowth spectrum

Day 60 opinion

Action: For adoption

Other

2.3.18. 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 60 opinion

Action: For adoption

Other

Note: Withdrawal request received on 29 October 2021

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

Ipsen Pharma; Treatment of fibrodysplasia ossificans progressiva

Day 60 opinion

Action: For adoption

Other

2.3.20. Synthetic double-stranded siRNA oligonucleotide directed against lactate dehydrogenase A mRNA and containing four modified nucleosides which form a ligand cluster of four N-acetylgalactosamine residues (DCR-PHXC, nedosiran) - Orphan - EMEA-002493-PIP01-18-M03

Dicerna Ireland Limited; Primary hyperoxaluria

Day 60 opinion

Action: For adoption

Uro-nephrology

2.4. Opinions on Re-examinations

2.4.1. Perflubutane - EMEA-003037-PIP01-21

GE Healthcare AS; Diagnostic evaluation of focal hepatic lesions

Day 30 opinion

Action: For adoption. Oral explanation to be held on Wednesday, 10 November at 14:00

Diagnostic / Oncology

2.4.2. Single-stranded, 5'-capped messenger RNA (mRNA) produced using a cell-free in vitro transcription from the corresponding DNA templates, encoding the viral spike (S) protein of SARS-CoV-2 - EMEA-002893-PIP01-20-M01

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

Day 30 opinion

Action: For adoption

Vaccines

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. Finerenone - EMEA-C2-001623-PIP01-14-M04

Bayer AG; Treatment of chronic kidney disease

Day 30 letter

Action: For information

Uro-nephrology

2.7.2. Sparsentan - EMEA-C1-001984-PIP02-20

Travere Therapeutics Ireland Limited; Treatment of focal segmental glomerular sclerosis (FSGS)

Day 30 letter

Action: For information

Uro-nephrology

3. Discussion of applications

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

3.1. Discussions on Products D90-D60-D30

3.1.1. EMEA-002958-PIP01-21

Treatment of hypertrophic cardiomyopathy

Day 90 discussion

Action: For discussion

Cardiovascular Diseases

3.1.2. Seralutinib - Orphan - EMEA-002972-PIP01-21

Gossamer Bio 002 Limited; Treatment of pulmonary arterial hypertension

Day 90 discussion

Action: For discussion

Cardiovascular Diseases

3.1.3. Single strain of non-genetically modified *Prevotella histicola* - EMEA-002933-PIP01-20

Treatment of psoriasis

Day 90 discussion

Action: For discussion

Dermatology

3.1.4. EMEA-002992-PIP01-21

Treatment of Fibrodysplasia Ossificans Progressiva (FOP)

Day 90 discussion

Action: For discussion

Endocrinology-Gynaecology-Fertility-Metabolism

3.1.5. Ethinyl estradiol / dienogest - EMEA-002229-PIP02-21

Treatment of polycystic ovary syndrome

Day 90 discussion

Action: For discussion

Endocrinology-Gynaecology-Fertility-Metabolism

3.1.6. Poly(oxy-1,2-ethanediyl), alpha-hydro-omega-methoxy, ether with N-[[[2-[[6-[[1-[3-[[3-(2,3-dihydroxypropoxy)propyl]amino]-3-oxopropyl]-2,5-dioxo-3-pyrrolidinyl]thio]hexyl]amino]ethyl]amino]carbonyl]-2-methylalanyl-teriparatide (2:1) - Orphan - EMEA-002955-PIP01-21

Ascendis Pharma Bone Diseases A/S; Treatment of hypoparathyroidism

Day 90 discussion

Action: For discussion

Endocrinology-Gynaecology-Fertility-Metabolism

3.1.7. Tildacerfont - Orphan - EMEA-002970-PIP01-21

Spruce Biosciences, Inc.; Treatment of congenital adrenal hyperplasia

Day 90 discussion

Action: For discussion

Endocrinology-Gynaecology-Fertility-Metabolism

3.1.8. Benralizumab - EMEA-001214-PIP07-21

Treatment of eosinophilic gastritis/eosinophilic gastroenteritis

Day 90 discussion

Action: For discussion

Gastroenterology-Hepatology

3.1.9. Izencitinib - EMEA-002757-PIP02-21

Treatment of Crohn's disease

Day 90 discussion

Action: For discussion

Gastroenterology-Hepatology

3.1.10. 6-[(3S,4S)-4-methyl-1-(pyrimidin-2-ylmethyl)pyrrolidin-3-yl]-3-tetrahydropyran-4-yl-7H-imidazo[1,5-a]pyrazin-8-one - Orphan - EMEA-002991-PIP01-21

IMARA Inc; Treatment of sickle cell disease

Day 90 discussion

Action: For discussion

Haematology-Hemostaseology

3.1.11. Benralizumab - EMEA-001214-PIP04-19

Treatment of hypereosinophilic syndrome (HES)

Day 90 discussion

Action: For discussion

Haematology-Hemostaseology

3.1.12. Alectinib - EMEA-002431-PIP02-21

Treatment of malignant neoplasms (except haematopoietic and lymphoid tissue neoplasms)

Day 90 discussion

Action: For discussion

Oncology

3.1.13. Nirogacestat hydrobromide - Orphan - EMEA-002971-PIP01-21

SpringWorks Therapeutics, Inc; Treatment of desmoid tumours

Day 90 discussion

Action: For discussion

Oncology

3.1.14. Ribociclib - EMEA-002765-PIP02-21

Treatment of neuroblastoma

Day 90 discussion

Action: For discussion

Oncology

3.1.15. Vorasidenib - EMEA-002932-PIP02-21

Treatment of glioma

Day 90 discussion

Action: For discussion

Oncology

3.1.16. EMEA-003002-PIP01-21

Treatment of proteinuric chronic kidney disease

Day 90 discussion

Action: For discussion

Uro-nephrology

3.1.17. ExPEC9V - EMEA-002996-PIP01-21

Prevention of *E.coli* infections / Prevention of infections caused by extraintestinal pathogenic *Escherichia coli* (ExPEC)

Day 90 discussion

Action: For discussion

Vaccines

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

Genzyme Europe B.V.; Treatment of Fabry disease

Day 60 discussion

Action: For discussion

Endocrinology-Gynaecology-Fertility-Metabolism

3.1.19. Resmetirom - EMEA-003087-PIP01-21

Treatment of non-alcoholic steatohepatitis (NASH)

Day 60 discussion

Action: For discussion

Gastroenterology-Hepatology

3.1.20. Ritlecitinib - EMEA-002451-PIP02-21

Treatment of ulcerative colitis

Day 60 discussion

Action: For discussion

Gastroenterology-Hepatology

3.1.21. EMEA-003090-PIP01-21

Hereditary angioedema

Day 60 discussion

Action: For discussion

Haematology-Hemostaseology

3.1.22. Deucravacitinib - EMEA-002350-PIP04-21

Treatment of ulcerative colitis

Day 60 discussion

Action: For discussion

Immunology-Rheumatology-Transplantation

3.1.23. Humanised IgG1K monoclonal antibody against interferon beta - Orphan - EMEA-003089-PIP01-21

Pfizer Europe MA EEIG; Treatment of Dermatomyositis

Day 60 discussion

Action: For discussion

Immunology-Rheumatology-Transplantation / Dermatology

3.1.24. EMEA-003081-PIP01-21

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

Day 60 discussion

Action: For discussion

Infectious Diseases

3.1.25. Bepirovirsen - EMEA-003082-PIP01-21

Treatment of chronic hepatitis B infection

Day 60 discussion

Action: For discussion

Infectious Diseases

3.1.26. Emvododstat - EMEA-003088-PIP01-21

Treatment of coronavirus disease 2019 (COVID-19)

Day 60 discussion

Action: For discussion

Infectious Diseases

3.1.27. Lonafarnib - Orphan - EMEA-002516-PIP02-21

EigerBio Europe Limited; Treatment of hepatitis D virus infection

Day 60 discussion

Action: For discussion

Infectious Diseases

3.1.28. Molnupiravir - EMEA-002940-PIP02-21

Prevention of coronavirus disease 2019 (COVID-19)

Day 60 discussion

Action: For discussion

Infectious Diseases

3.1.29. Plitidepsin - Orphan - EMEA-000095-PIP02-21

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

Day 60 discussion

Action: For discussion

Infectious Diseases

3.1.30. *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 60 discussion

Action: For discussion

Neurology

Note: Withdrawal request received on 20 October 2021

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

Treatment of Hodgkin lymphoma

Day 60 discussion

Action: For discussion

Oncology

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

Treatment of solid tumours

Day 60 discussion

Action: For discussion

Oncology

3.1.33. Benralizumab - EMEA-001214-PIP09-21

Treatment of eosinophilic granulomatosis with polyangiitis (EGPA)

Day 60 discussion

Action: For discussion

Pneumology - Allergology

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

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

Day 60 discussion

Action: For discussion

Uro-nephrology

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

Prevention of coronavirus disease 2019 (COVID-19)

Day 60 discussion

Action: For discussion

Vaccines

3.1.36. Acetylsalicylic acid / rosuvastatin calcium - EMEA-002239-PIP02-21

Prevention of cardiovascular events

Day 30 discussion

Action: For discussion

Cardiovascular Diseases

3.1.37. Colchicine - EMEA-003101-PIP01-21

Reduction of atherothrombic events in patients with coronary artery disease

Day 30 discussion

Action: For discussion

Cardiovascular Diseases

3.1.38. Derivative of pyrrolopyrimidine - EMEA-003109-PIP01-21

Heart failure with LVEF > 40% / Prevention of CV outcome events in patients with HF with LVEF > 40%

Day 30 discussion

Action: For discussion

Cardiovascular Diseases

3.1.39. Insulin efsitora alfa - EMEA-003105-PIP01-21

Treatment of type 1 diabetes mellitus / Treatment of type 2 diabetes mellitus

Day 30 discussion

Action: For discussion

Endocrinology-Gynaecology-Fertility-Metabolism

3.1.40. Pudexacianinium - EMEA-003099-PIP01-21

Ureter visualisation

Day 30 discussion

Action: For discussion

Endocrinology-Gynaecology-Fertility-Metabolism / Diagnostic / Oncology / Gastroenterology-Hepatology / Uro-nephrology

3.1.41. Efruxifermin - EMEA-003114-PIP01-21

Treatment of non-alcoholic fatty liver disease including non-alcoholic steatohepatitis

Day 30 discussion

Action: For discussion

Gastroenterology-Hepatology

3.1.42. Omfiloctocog alfa - EMEA-003113-PIP01-21

Control and prevention of bleeding / Perioperative management

Day 30 discussion

Action: For discussion

Haematology-Hemostaseology

3.1.43. Cenerimod - EMEA-003108-PIP01-21

Treatment of systemic lupus erythematosus (SLE)

Day 30 discussion

Action: For discussion

Immunology-Rheumatology-Transplantation

3.1.44. Fostamatinib - EMEA-001196-PIP03-21

Treatment of autoimmune haemolytic anaemia

Day 30 discussion

Action: For discussion

Immunology-Rheumatology-Transplantation / Haematology-Hemostaseology

3.1.45. Adeno-associated virus serotype hu68 containing the human GLB1 gene - Orphan - EMEA-003102-PIP01-21

Passage Bio, Inc.; Treatment of GM1 gangliosidosis

Day 30 discussion

Action: For discussion

Neurology

3.1.46. Corticotropin - EMEA-003097-PIP01-21

Treatment of infantile spasms

Day 30 discussion

Action: For discussion

Neurology

3.1.47. Gantenerumab - EMEA-003107-PIP01-21

Alzheimer's disease

Day 30 discussion

Action: For discussion

Neurology

3.1.48. Humanised monoclonal IgG1-based antibody - EMEA-003100-PIP01-21

Treatment of spinal muscular atrophy

Day 30 discussion

Action: For discussion

Neurology

3.1.49. Aumolertinib - EMEA-003106-PIP01-21

Treatment of lung cancer

Day 30 discussion

Action: For discussion

Oncology

3.1.50. Pembrolizumab / favezelimab - EMEA-003104-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 30 discussion

Action: For discussion

Oncology

3.1.51. Triazolopyrimidine derivative - EMEA-003095-PIP01-21

Diabetic retinopathy / Treatment of diabetic retinopathy

Day 30 discussion

Action: For discussion

Ophthalmology

3.1.52. Amifampridine - EMEA-003103-PIP01-21

Lambert-Eaton myasthenic syndrome

Day 30 discussion

Action: For discussion

Other

3.1.53. 2'-O-(2'-methoxyethyl) modified antisense oligonucleotide targeting prekallikrein (PKK) mRNA - EMEA-003112-PIP01-21

Hereditary angioedema / Prevention of hereditary angioedema

Day 30 discussion

Action: For discussion

Pneumology - Allergology / Haematology-Hemostaseology

3.1.54. Troriluzole - EMEA-003084-PIP02-21

Treatment of obsessive-compulsive disorder

Day 30 discussion

Action: For discussion

Psychiatry

3.1.55. EMEA-003098-PIP01-21

Treatment of proteinuric chronic kidney disease

Day 30 discussion

Action: For discussion

Uro-nephrology

3.1.56. Bardoxolone - EMEA-002488-PIP02-21

Treatment of autosomal dominant polycystic kidney disease (ADPKD)

Day 30 discussion

Action: For discussion

Uro-nephrology

3.1.57. Pegcetacoplan - Orphan - EMEA-002600-PIP03-21

Apellis Ireland Limited; Treatment of glomerulonephritis and nephrotic syndrome

Day 30 discussion

Action: For discussion

Uro-nephrology

3.1.58. Vibegron - EMEA-001415-PIP02-21

Treatment of myoneurogenic bladder disorders

Day 30 discussion

Action: For discussion

Uro-nephrology

3.1.59. Respiratory syncytial virus stabilised prefusion F subunit vaccine (RSVpreF) - EMEA-002795-PIP02-21

Prevention of RSV-associated lower respiratory tract illness

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. Tralokinumab - EMEA-C2-001900-PIP02-17-M05

LEO Pharma A/S; Treatment of atopic dermatitis

Day 30 discussion

Action: For discussion

Dermatology

3.2.2. PEGylated-fibroblast growth factor 21 - EMEA-C1-002448-PIP01-18-M02

Bristol-Myers Squibb International Corporation; Treatment of non-alcoholic steatohepatitis (NASH)

Day 30 discussion

Action: For discussion

Gastroenterology-Hepatology

3.2.3. Simeticone / macrogol 4000 / potassium chloride / sodium sulphate, anhydrous / sodium chloride / citric acid, anhydrous / sodium citrate - EMEA-C-001356-PIP02-12-M04

Alfasigma S.p.A.; Bowel cleansing prior to clinical procedures

Day 30 discussion

Action: For discussion

Gastroenterology-Hepatology

3.2.4. Peramivir - EMEA-C-001856-PIP02-16-M02

BioCryst Ireland Limited; Treatment of influenza

Day 30 discussion

Action: For discussion

Infectious Diseases

3.2.5. Nivolumab - EMEA-C3-001407-PIP02-15-M05

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

Day 30 discussion

Action: For discussion

Oncology

3.2.6. Selpercatinib - EMEA-C2-002544-PIP01-18-M01

Eli Lilly and Company; 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. Pneumococcal polysaccharide serotype 1 – diphtheria CRM197 conjugate / pneumococcal polysaccharide serotype 3 – diphtheria CRM197 conjugate / pneumococcal polysaccharide serotype 4 – diphtheria CRM197 conjugate / pneumococcal polysaccharide serotype 5 – diphtheria CRM197 conjugate / pneumococcal polysaccharide serotype 6A – diphtheria CRM197 conjugate / pneumococcal polysaccharide serotype 6B – diphtheria CRM197 conjugate / pneumococcal polysaccharide serotype 7F – diphtheria CRM197 conjugate / pneumococcal polysaccharide serotype 9V – diphtheria CRM197 conjugate / pneumococcal polysaccharide serotype 14 – diphtheria CRM197 conjugate / pneumococcal polysaccharide serotype 18C – diphtheria CRM197 conjugate / pneumococcal polysaccharide serotype 19A – diphtheria CRM197 conjugate / pneumococcal polysaccharide serotype 19F – diphtheria CRM197 conjugate /

pneumococcal polysaccharide serotype 22F – diphtheria CRM197 conjugate / pneumococcal polysaccharide serotype 23F – diphtheria CRM197 conjugate / pneumococcal polysaccharide serotype 33F – diphtheria CRM197 conjugate (15-valent pneumococcal polysaccharide conjugate vaccine [V114]) - EMEA-C-002215-PIP01-17-M03

Merck Sharp & Dohme (Europe), Inc.; Prevention of disease caused by *Streptococcus pneumoniae*

Day 30 discussion

Action: For discussion

Vaccines

3.3. Discussions on Modification of an Agreed Paediatric Investigation Plan

3.3.1. Dupilumab - EMEA-001501-PIP02-13-M07

sanofi-aventis groupe; Treatment of asthma

Day 30 discussion

Action: For discussion

Dermatology

3.3.2. Mitapivat - Orphan - EMEA-002684-PIP01-19-M01

Agios Netherlands B.V.; Pyruvate kinase deficiency

Day 30 discussion

Action: For discussion

Haematology-Hemostaseology

3.3.3. Vadadustat - EMEA-001944-PIP01-16-M03

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

Day 30 discussion

Action: For discussion

Haematology-Hemostaseology

3.3.4. Allogeneic bone marrow derived mesenchymal stromal cells, *ex-vivo* expanded - Orphan - EMEA-002706-PIP01-19-M01

medac Gesellschaft für klinische Spezialpräparate mbH; Treatment of acute graft-versus-host disease

Day 30 discussion

Action: For discussion

Immunology-Rheumatology-Transplantation

3.3.5. Apremilast - EMEA-000715-PIP03-11-M07

Amgen Europe B.V.; Treatment of psoriasis

Day 30 discussion

Action: For discussion

Immunology-Rheumatology-Transplantation

3.3.6. Baricitinib - EMEA-001220-PIP01-11-M06

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

Day 30 discussion

Action: For discussion

Immunology-Rheumatology-Transplantation

3.3.7. Avibactam / ceftazidime - EMEA-001313-PIP01-12-M11

Pfizer Europe MA EEIG; Treatment of bacterial infections

Day 30 discussion

Action: For discussion

Infectious Diseases

3.3.8. Cabotegravir - EMEA-001418-PIP01-13-M04

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

Day 30 discussion

Action: For discussion

Infectious Diseases

3.3.9. Cobicistat / atazanavir sulphate - EMEA-001465-PIP01-13-M04

Bristol-Myers Squibb Pharma EEIG; Treatment of human immunodeficiency virus (HIV-1) infection

Day 30 discussion

Action: For discussion

Infectious Diseases

3.3.10. Tenofovir alafenamide / rilpivirine / emtricitabine - EMEA-001679-PIP01-14-M01

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

Day 30 discussion

Action: For discussion

Infectious Diseases

3.3.11. Brivaracetam - Orphan - EMEA-000332-PIP02-17-M03

UCB Pharma S.A.; Treatment of neonatal seizures / Treatment of paediatric epilepsy syndromes

Day 30 discussion

Action: For discussion

Neurology

3.3.12. Onasemnogene abeparvovec - Orphan - EMEA-002168-PIP01-17-M04

Novartis Gene Therapy EU Limited; Treatment of spinal muscular atrophy

Day 30 discussion

Action: For discussion

Neurology

3.3.13. Phenobarbital - EMEA-002532-PIP01-18-M02

Proveca Pharma Limited; Epilepsy

Day 30 discussion

Action: For discussion

Neurology

3.3.14. Avapritinib - Orphan - EMEA-002358-PIP02-18-M02

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

Day 30 discussion

Action: For discussion

Oncology

3.3.15. Gemtuzumab ozogamicin - Orphan - EMEA-001733-PIP02-15-M02

Pfizer Europe MA EEIG; Treatment of acute myloid leukemia

Day 30 discussion

Action: For discussion

Oncology

3.3.16. Talimogene laherparepvec - EMEA-001251-PIP01-11-M05

Amgen Europe B.V.; Treatment of solid malignant non-CNS tumours

Day 30 discussion

Action: For discussion

Oncology

3.3.17. Cysteamine - Orphan - EMEA-000322-PIP01-08-M06

Recordati Rare Diseases SARL; Cystinosis

Day 30 discussion

Action: For discussion

Ophthalmology

3.3.18. Lanadelumab - Orphan - EMEA-001864-PIP01-15-M06

Takeda Pharmaceuticals International AG Ireland Branch; Hereditary angioedema

Day 30 discussion

Action: For discussion

Other

3.3.19. Budesonide / glycopyrronium bromide / formoterol fumarate dihydrate - EMEA-002063-PIP01-16-M01

AstraZeneca AB; Asthma

Day 30 discussion

Action: For discussion

Pneumology - Allergology

3.3.20. Dexmedetomidine hydrochloride, Oromucosal Film - EMEA-002758-PIP01-19-M01

BioXcel Therapeutics, Inc.; Acute agitation in bipolar disorder / Acute agitation in schizophrenia

Day 30 discussion

Action: For discussion

Psychiatry

3.3.21. COVID-19 Vaccine (ChAdOx1-S [recombinant]) - EMEA-002862-PIP01-20-M02

AstraZeneca AB; Prevention of coronavirus disease 2019 (COVID-19)

Day 30 discussion

Action: For discussion

Vaccines

4. Nominations

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

4.1. List of submissions of applications with start of procedure 22 November 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. Tramadol hydrochloride / magnesium lactate dihydrate- EMEA-12-2021

SciencePharma spółka z ograniczoną odpowiedzialnością spółka jawna; All classes of medicinal products for treatment of primary and secondary osteoarthrosis; Management of chronic pain in adults with osteoarthritis of the hip and/or knee.

Action: For adoption

6.1.2. Tozorakimab - EMEA-14-2021

AstraZeneca AB; All classes of medicinal products for the treatment of chronic obstructive pulmonary disease (COPD) (excluding chronic lung diseases associated with long-term airflow limitation, such as asthma, bronchopulmonary dysplasia, primary cilia dyskinesia, obstructive lung disease related to graft versus-host disease after [bone-marrow] transplantation); Treatment of symptomatic COPD in patients with a history of exacerbations

Action: For adoption

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

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

No item

8. Annual reports on deferrals

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

9. Organisational, regulatory and methodological matters

9.1. Mandate and organisation of the PDCO

9.1.1. PDCO membership

Action: For information

9.1.2. Vote by proxy

Action: For information

9.2. 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

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

Meeting Summary PCWP-HCPWP joint meeting on 21 - 22 September 2021

Draft Agenda - Annual PCWP-HCPWP joint meeting with all Eligible Organisations on 24 November 2021

Action: For information

9.4. Cooperation within the EU regulatory network

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

Action: For information

9.5. Cooperation with International Regulators

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

No item

9.8. Planning and reporting

No item

10. Any other business

10.1. COVID-19 update

Action: For information

10.2. International Council for Harmonisation – ICH E11A – Pediatric Extrapolation

Action: For information

10.3. Real World Evidence pilot with PDCO

Action: For information

10.4. R&D focus group 'evolutionary PIP'

Action: For information

10.5. New operational model on Working Parties - update

Action: For information

11. Breakout sessions

11.1. Internal PDCO Operations

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

11.2. Neonatology

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

11.3. Paediatric oncology

Action: For discussion on Wednesday, 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/