



EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

18 May 2021
EMA/PDCO/246845/2021
Human Medicines Division

Paediatric Committee (PDCO)

Draft Agenda for the meeting on 18-21 May 2021

Chair: Koenraad Norga – Vice-Chair: Sabine Scherer

18 May 2021, 10:00- 17:00

19 May 2021, 08:30- 19:00

20 May 2021, 08:30- 19:00

21 May 2021, 08:30- 13:00

Health and safety information

In accordance with the Agency's health and safety policy, delegates are to be briefed on health, safety and emergency information and procedures prior to the start of the meeting.

Disclaimers

Some of the information contained in this agenda is considered commercially confidential or sensitive and therefore not disclosed. With regard to intended therapeutic indications or procedure scopes listed against products, it must be noted that these may not reflect the full wording proposed by applicants and may also vary during the course of the review. Additional details on some of these procedures will be published in the PDCO Committee meeting reports (after the PDCO Opinion is adopted), and on the Opinions and decisions on paediatric investigation plans webpage (after the EMA Decision is issued).

Note on access to documents

Some documents mentioned in the agenda cannot be released at present following a request for access to documents within the framework of Regulation (EC) No 1049/2001 as they are subject to 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 18-21 May 2021. See 18-21 May 2021 PDCO minutes (to be published post June 2021 PDCO meeting).

1.2. Adoption of agenda

PDCO agenda for 18-21 May 2021

1.3. Adoption of the minutes

PDCO minutes for 20-23 April 2021

PDCO extraordinary meeting minutes for 9 April 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. Finerenone - EMEA-001623-PIP03-20

Treatment of heart failure

Day 120 opinion

Action: For adoption

Cardiovascular Diseases

2.1.2. Macitentan - Orphan - EMEA-001032-PIP03-19

Janssen-Cilag International N.V.; Treatment of functional univentricular heart disease with total cavo-pulmonary connection

Day 120 opinion

Action: For adoption

Cardiovascular Diseases

2.1.3. Ralinepag - Orphan - EMEA-002432-PIP02-20

United Therapeutics Corporation; Treatment of pulmonary arterial hypertension

Day 120 opinion

Action: For adoption

Cardiovascular Diseases

2.1.4. Allogeneic skin-derived ABCB5-positive mesenchymal stem cells - Orphan - EMEA-002875-PIP01-20

RHEACELL GmbH & Co. KG; Treatment of epidermolysis bullosa

Day 120 opinion

Action: For adoption

Dermatology

2.1.5. Adeno-associated viral vector serotype 8 containing the human glucose-6-phosphatase gene (DTX401) - Orphan - EMEA-002734-PIP01-19

Ultragenyx Germany GmbH; Glycogen storage disease type Ia / Treatment of glycogen storage disease type Ia

Day 120 opinion

Action: For adoption

Endocrinology-Gynaecology-Fertility-Metabolism

2.1.6. Pyridoxal 5'-phosphate monohydrate - Orphan - EMEA-002404-PIP01-18

Medicure Pharma Europe Limited; Pyridox(am)ine 5'-phosphate oxidase (PNPO) deficiency

Day 120 opinion

Action: For adoption

Endocrinology-Gynaecology-Fertility-Metabolism

Note: Withdrawal request received on 7 May 2021

2.1.7. Maralixibat Chloride - Orphan - EMEA-001475-PIP04-20

Mirum Pharmaceuticals Inc.; Treatment of biliary atresia

Day 120 opinion

Action: For adoption

Gastroenterology-Hepatology

2.1.8. Odevixibat - Orphan - EMEA-002054-PIP03-20

Albireo AB; Treatment of Alagille syndrome

Day 120 opinion

Action: For adoption

Gastroenterology-Hepatology

2.1.9. Recombinant monoclonal antibody to sialic acid-binding Ig-like lectin 8 - Orphan - EMEA-002856-PIP01-20

Allakos Inc; Treatment of eosinophilic gastrointestinal inflammatory disorders

Day 120 opinion

Action: For adoption

Gastroenterology-Hepatology

2.1.10. Cilgavimab - EMEA-002925-PIP01-20

Prevention or treatment of COVID-19

Day 120 opinion

Action: For adoption

Infectious Diseases

2.1.11. Tixagevimab - EMEA-002900-PIP01-20

Prevention or treatment of COVID-19

Day 120 opinion

Action: For adoption

Infectious Diseases

2.1.12. Pralsetinib - EMEA-002575-PIP02-20

Treatment of all conditions included in the category of malignant neoplasms (except haematopoietic and lymphoid tissue neoplasms) / Treatment of thyroid neoplasms

Day 120 opinion

Action: For adoption

Oncology

2.1.13. Autologous selected renal cells - EMEA-002844-PIP01-20

Treatment of chronic kidney disease

Day 120 opinion

Action: For adoption

Uro-nephrology

2.1.14. Selatogrel - EMEA-002967-PIP01-21

Treatment of acute myocardial infarction (AMI)

Day 60 opinion

Action: For adoption

Cardiovascular Diseases

2.1.15. Vupanorsen - EMEA-002973-PIP01-21

Treatment of hypertriglyceridaemia / Prevention of cardiovascular events

Day 60 opinion

Action: For adoption

Cardiovascular Diseases

2.1.16. Mannitol - EMEA-002968-PIP01-21

Bowel cleansing prior to clinical procedure

Day 60 opinion

Action: For adoption

Gastroenterology-Hepatology

2.1.17. Anti-C1s Humanized IgG4 Monoclonal Antibody - EMEA-002903-PIP02-21

Treatment of cold agglutinin disease

Day 60 opinion

Action: For adoption

Immunology-Rheumatology-Transplantation / Haematology-Hemostaseology / Neurology

2.1.18. EMEA-002975-PIP01-21

Visualisation of prostate specific membrane antigen (PSMA) overexpressing tumors

Day 60 opinion

Action: For adoption

Oncology

2.1.19. Datopotamab deruxtecan - EMEA-002976-PIP01-21

Treatment of non-small cell lung cancer

Day 60 opinion

Action: For adoption

Oncology

2.1.20. Human Papilloma Virus Type 16 E6 001-032/Human Papilloma Virus Type 16 E6 019-050/Human Papilloma Virus Type 16 E6 041-065/Human Papilloma Virus Type 16 E6 055-080/Human Papilloma Virus Type 16 E6 085-109/Human Papilloma Virus Type 16 E6 091-122/Human Papilloma Virus Type 16 E6 127-158 / Human Papilloma Virus Type 16 E6 071-095/Human Papilloma Virus Type 16 E6 109-140/Human Papilloma Virus Type 16 E7 001-035/Human Papilloma Virus Type 16 E7 022-056/Human Papilloma Virus Type 16 E7 064-098 - EMEA-001055-PIP02-21

Treatment of HPV16 positive malignancies

Day 60 opinion

Action: For adoption

Oncology

2.1.21. Patritumab deruxtecan - EMEA-002977-PIP01-21

Treatment of non-small cell lung cancer

Day 60 opinion

Action: For adoption

Oncology

2.1.22. Trastuzumab deruxtecan - EMEA-002978-PIP01-21

Treatment of non-small cell lung cancer

Day 60 opinion

Action: For adoption

Oncology

2.1.23. Dapagliflozin propanediol monohydrate / Zibotentan - EMEA-002969-PIP01-21

Treatment of chronic kidney disease

Day 60 opinion

Action: For adoption

Other

2.1.24. Regdanvimab - EMEA-002961-PIP01-21

Treatment of coronavirus disease 2019 (COVID-19)

Day 90 opinion

Action: For adoption

Infectious Diseases

2.1.25. Fully human neutralizing immunoglobulin G-1 kappa monoclonal antibody directed against a conserved epitope on the SARS CoV1 and 2 spike protein - EMEA-002899-PIP01-20

Treatment of coronavirus disease 2019 (COVID-19)

Day 90 opinion

Action: For discussion

Infectious Diseases

2.2. Opinions on Compliance Check

No items

2.3. Opinions on Modification of an Agreed Paediatric Investigation Plan

2.3.1. Nemolizumab - EMEA-001624-PIP01-14-M03

Galderma International S.A.; Atopic dermatitis

Day 60 opinion

Action: For adoption

Dermatology

2.3.2. Bis-choline tetrathiomolybdate - Orphan - EMEA-002232-PIP02-19-M01

Alexion Europe S.A.S.; Treatment of Wilson Disease

Day 60 opinion

Action: For adoption

Endocrinology-Gynaecology-Fertility-Metabolism

2.3.3. Canagliflozin (1s)-1,5-anhydro-1-[3-[[5-(4-fluorophenyl)-2-thienyl]methyl]-4-methylphenyl]-D-glucitol hemihydrate - EMEA-001030-PIP01-10-M09

Janssen-Cilag International NV; Type 2 diabetes mellitus

Day 60 opinion

Action: For adoption
Endocrinology-Gynaecology-Fertility-Metabolism

2.3.4. [Cotadutide - EMEA-002287-PIP01-17-M02](#)

AstraZeneca AB; Treatment of Type 2 diabetes mellitus
Day 60 opinion

Action: For adoption
Endocrinology-Gynaecology-Fertility-Metabolism

2.3.5. [Romosozumab - EMEA-001075-PIP04-15-M03](#)

UCB Pharma S.A.; Treatment of osteoporosis
Day 60 opinion

Action: For adoption
Endocrinology-Gynaecology-Fertility-Metabolism

2.3.6. [Eluxadoline - EMEA-001579-PIP01-13-M04](#)

Allergan Pharmaceuticals International Limited; Treatment of diarrhoea-predominant irritable bowel syndrome
Day 60 opinion

Action: For adoption
Gastroenterology-Hepatology

2.3.7. [Ozanimod hydrochloride - EMEA-001710-PIP03-17-M03](#)

Celgene Europe B.V.; Treatment of ulcerative colitis
Day 60 opinion

Action: For adoption
Gastroenterology-Hepatology

2.3.8. [Garadacimab - EMEA-002726-PIP01-19-M01](#)

CSL Behring GmbH; Prevention of hereditary angioedema attacks
Day 60 opinion

Action: For adoption
Haematology-Hemostaseology

2.3.9. Cotadutide - EMEA-002712-PIP01-19-M01

AstraZeneca AB; Treatment of non-alcoholic steatohepatitis (NASH)

Day 60 opinion

Action: For adoption

Immunology-Rheumatology-Transplantation

2.3.10. Guselkumab - EMEA-001523-PIP03-18-M01

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

Day 60 opinion

Action: For adoption

Immunology-Rheumatology-Transplantation

2.3.11. Rilzabrutinib - Orphan - EMEA-002438-PIP02-19-M01

Principia Biopharma, Inc.; Treatment of immune thrombocytopenia

Day 60 opinion

Action: For adoption

Immunology-Rheumatology-Transplantation

2.3.12. Cobicistat / darunavir - EMEA-001280-PIP01-12-M04

Janssen-Cilag International NV; Treatment of HIV-1 infection

Day 60 opinion

Action: For adoption

Infectious Diseases

2.3.13. Dolutegravir (DTG) - EMEA-000409-PIP01-08-M06

ViiV Healthcare UK Limited; Treatment of Human Immunodeficiency Virus Type 1 (HIV-1) infection

Day 60 opinion

Action: For adoption

Infectious Diseases

2.3.14. Ibalizumab - EMEA-002311-PIP01-17-M02

Theratechnologies Europe Limited; Treatment of Human Immunodeficiency Virus Type 1 (HIV-1) infection

Day 60 opinion

Action: For adoption

Infectious Diseases

2.3.15. Oseltamivir phosphate - EMEA-000365-PIP01-08-M12

Roche Registration GmbH; Treatment and prevention of influenza

Day 60 opinion

Action: For adoption

Infectious Diseases

2.3.16. Oteseconazole - EMEA-002392-PIP01-18-M01

Gedeon Richter Plc.; ICD-10 Code B373, treatment of vulvovaginal candidiasis

Day 60 opinion

Action: For adoption

Infectious Diseases

2.3.17. Peramivir - EMEA-001856-PIP02-16-M02

BioCryst Ireland Limited; Treatment of influenza

Day 60 opinion

Action: For adoption

Infectious Diseases

2.3.18. Tenofovir alafenamide - EMEA-001584-PIP01-13-M06

Gilead Sciences International Ltd.; Treatment of chronic viral hepatitis B

Day 60 opinion

Action: For adoption

Infectious Diseases

2.3.19. Vaborbactam / meropenem - EMEA-001731-PIP01-14-M03

Menarini International Operations Luxembourg S.A.; Treatment of Gram-negative bacterial infections

Day 60 opinion

Action: For adoption

Infectious Diseases

2.3.20. Nivolumab - EMEA-001407-PIP02-15-M05

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

Day 60 opinion

Action: For adoption

Oncology

2.3.21. Lanadelumab - Orphan - EMEA-001864-PIP01-15-M05

Shire Pharmaceuticals Ireland Limited; Treatment of hereditary angioedema

Day 60 opinion

Action: For adoption

Other

2.3.22. Ravulizumab (ALXN1210) - Orphan - EMEA-001943-PIP01-16-M06

Alexion Europe SAS; Treatment of atypical haemolytic uremic syndrome

Day 60 opinion

Action: For adoption

Uro-nephrology / Haematology-Hemostaseology

2.3.23. Ravulizumab (ALXN1210) - Orphan - EMEA-002077-PIP01-16-M04

Alexion Europe SAS; Paroxysmal nocturnal haemoglobinuria

Day 60 opinion

Action: For adoption

Uro-nephrology / Haematology-Hemostaseology

2.3.24. COVID-19 Vaccine (ChAdOx1-S [recombinant]) - EMEA-002862-PIP01-20-M01

AstraZeneca AB; COVID-19 vaccine is indicated for active immunisation to prevent COVID-19 caused by SARS-CoV-2

Day 60 opinion

Action: For adoption

Vaccines

2.4. Opinions on Re-examinations

No items

2.5. Opinions on Review of Granted Waivers

No items

2.6. Finalisation and adoption of Opinions

No items

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. Crizotinib - EMEA-C2-001493-PIP03-18-M01

Pfizer Europe MA EEIG; Treatment of inflammatory myofibroblastic tumour

Day 1 letter

Action: For information

Oncology

2.7.2. Valoctocogene roxaparvovec - EMEA-C2-002427-PIP01-18-M01

BioMarin International Ltd.; Treatment of Haemophilia A

Day 1 letter

Action: For information

Haematology-Hemostaseology

2.7.3. Tenofovir (disoproxil fumarate) - EMEA-C5-000533-PIP01-08-M10

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

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. Human, recombinant, non-fucosylated IgG1k monoclonal antibody targeting OX-40 receptor on activated T cells - EMEA-002886-PIP01-20

Atopic dermatitis / Atopic Dermatitis

Day 90 discussion

Action: For discussion

Dermatology

3.1.2. Infigratinib - EMEA-002594-PIP02-20

Treatment of achondroplasia / Achondroplasia

Day 90 discussion

Action: For discussion

Endocrinology-Gynaecology-Fertility-Metabolism

3.1.3. (1R,3S,5R)-2-(2-(3-acetyl-5-(2-methylpyrimidin-5-yl)-1H-indazol-1-yl)acetyl)-N-(6-bromo-3-methylpyridin-2-yl)-5-methyl-2-azabicyclo[3.1.0]hexane-3-carboxamide - Orphan - EMEA-002863-PIP01-20

Alexion Europe SAS; Paroxysmal nocturnal haemoglobinuria

Day 90 discussion

Action: For discussion

Haematology-Hemostaseology

3.1.4. EMEA-002350-PIP03-20

Treatment of systemic lupus erythematosus (SLE)

Day 90 discussion

Action: For discussion

Immunology-Rheumatology-Transplantation

3.1.5. Fenebrutinib - EMEA-002349-PIP03-20

Treatment of multiple sclerosis / Multiple sclerosis

Day 90 discussion

Action: For discussion

Immunology-Rheumatology-Transplantation

3.1.6. Itraconazole - Orphan - EMEA-002787-PIP01-20

Laboratoires S.M.B. S.A.; Prevention of invasive mould disease

Day 90 discussion

Action: For discussion

Infectious Diseases / Oncology

3.1.7. Trimodulin - EMEA-002883-PIP01-20

Treatment of infections

Day 90 discussion

Action: For discussion

Neonatology - Paediatric Intensive Care / Infectious Diseases / Pneumology - Allergology

3.1.8. Zidebactam / cefepime - EMEA-002892-PIP01-20

Treatment of complicated urinary tract infections

Day 90 discussion

Action: For discussion

Neonatology - Paediatric Intensive Care / Infectious Diseases / Uro-nephrology

3.1.9. Ublituximab - EMEA-002889-PIP02-20

Relapsing forms of multiple sclerosis (RMS) / Treatment of multiple sclerosis

Day 90 discussion

Action: For discussion

Neurology

3.1.10. Vatiquinone - Orphan - EMEA-001238-PIP02-20

PTC Therapeutics International Limited; Treatment of mitochondrial epilepsy, excluding Leigh Syndrome / Treatment of mitochondrial disease

Day 90 discussion

Action: For discussion

Neurology

3.1.11. Afamitresgene autoleucel - Orphan - EMEA-002867-PIP01-20

Adaptimmune Ltd; Treatment of soft tissue sarcoma

Day 90 discussion

Action: For discussion

Oncology

3.1.12. Allogeneic anti-CD19 CAR T cells produced using CRISPR/Cas9 to disrupt the T cell receptor alpha constant (TRAC) and β 2-microglobulin (B2M) genomic loci and a recombinant adeno-associated viral vector to deliver donor template for insertion of the anti-CD19 CAR expression cassette into the TRAC locus - EMEA-002881-PIP01-20

Mature B cell neoplasms / Mature B cell neoplasms and B cell lymphoblastic leukemia/lymphoma / B cell lymphoblastic leukemia/lymphoma

Day 90 discussion

Action: For discussion

Oncology

3.1.13. Iptacopan - Orphan - EMEA-002705-PIP03-20

Novartis Europharm Limited; Paroxysmal nocturnal haemoglobinuria

Day 90 discussion

Action: For discussion

Other / Haematology-Hemostaseology

3.1.14. Bardoxolone methyl - Orphan - EMEA-002488-PIP01-18

Reata Pharmaceuticals Inc.; Treatment of Alport syndrome

Day 90 discussion

Action: For discussion

Uro-nephrology

3.1.15. SERALUTINIB - Orphan - EMEA-002972-PIP01-21

Gossamer Bio 002 Limited; Treatment of pulmonary arterial hypertension

Day 60 discussion

Action: For discussion

Cardiovascular Diseases

3.1.16. Ruxolitinib - EMEA-002618-PIP03-21

Atopic dermatitis

Day 60 discussion

Action: For discussion

Dermatology

3.1.17. Benzylamine derivative of benzofuran - EMEA-002974-PIP01-21

Treatment of paroxysmal nocturnal haemoglobinuria

Day 60 discussion

Action: For discussion

Haematology-Hemostaseology

3.1.18. Ravulizumab - EMEA-001943-PIP04-20

Aquaporin-4 antibody-positive neuromyelitis optica spectrum disorder

Day 60 discussion

Action: For discussion

Neurology

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

Roche Registration GmbH; Treatment of myasthenia gravis

Day 60 discussion

Action: For discussion

Neurology

3.1.20. Vatiquinone - EMEA-001238-PIP03-21

Treatment of Friedreich Ataxia

Day 60 discussion

Action: For discussion

Neurology

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

SpringWorks Therapeutics, Inc; Treatment of desmoid tumours

Day 60 discussion

Action: For discussion

Oncology

3.1.22. Ribociclib - EMEA-002765-PIP02-21

Treatment of neuroblastoma

Day 60 discussion

Action: For discussion

Oncology

3.1.23. 2'-O-(2-methoxyethyl) phosphorothioate antisense oligonucleotide targeting CD49d RNA - Orphan - EMEA-002981-PIP01-21

Antisense Therapeutics Limited; Treatment of Duchenne muscular dystrophy

Day 60 discussion

Action: For discussion

Other

3.1.24. Pamrevlumab - Orphan - EMEA-002979-PIP01-21

FibroGen, Inc.; Treatment of Duchenne Muscular Dystrophy

Day 60 discussion

Action: For discussion

Other

3.1.25. Evenamide - EMEA-002519-PIP03-21

Treatment of schizophrenia

Day 60 discussion

Action: For discussion

Psychiatry

3.1.26. 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 (serogroup B)

Day 60 discussion

Action: For discussion

Vaccines

3.1.27. Bentracimab - EMEA-002766-PIP02-21

Treatment of ticagrelor associated haemorrhage / Prevention of ticagrelor associated haemorrhage

Day 30 discussion

Action: For discussion

Cardiovascular Diseases

3.1.28. Hydrochlorothiazide / Amlodipine / Ramipril - EMEA-002998-PIP01-21

Treatment of hypertension

Day 30 discussion

Action: For discussion

Cardiovascular Diseases

3.1.29. Breprocitinib - EMEA-003011-PIP01-21

Treatment of atopic dermatitis

Day 30 discussion

Action: For discussion

Dermatology

3.1.30. Human Immunoglobulin G1 constant region – human ectodysplasin-A1 receptor-binding domain fusion protein - Orphan - EMEA-002995-PIP01-21

EspeRare Foundation; X-linked hypohidrotic ectodermal dysplasia (XLHED)

Day 30 discussion

Action: For discussion

Dermatology

3.1.31. Ulobetasol propionate - EMEA-003000-PIP01-21

Treatment of psoriasis / The proposed condition is moderate to severe plaque psoriasis. The proposed medicinal product is aimed as "treatment"

Day 30 discussion

Action: For discussion

Dermatology

3.1.32. EMEA-002992-PIP01-21

Treatment of Fibrodysplasia Ossificans Progressiva (FOP).

Day 30 discussion

Action: For discussion

Endocrinology-Gynaecology-Fertility-Metabolism

3.1.33. Empagliflozin - EMEA-000828-PIP07-21

Treatment of ischaemic heart disease

Day 30 discussion

Action: For discussion

Endocrinology-Gynaecology-Fertility-Metabolism

3.1.34. Ethinyl estradiol / Dienogest - EMEA-002229-PIP02-21

Treatment of polycystic ovary syndrome

Day 30 discussion

Action: For discussion

Endocrinology-Gynaecology-Fertility-Metabolism

3.1.35. Semaglutide - EMEA-001441-PIP06-21

Treatment of obesity

Day 30 discussion

Action: For discussion

Endocrinology-Gynaecology-Fertility-Metabolism

3.1.36. Semaglutide / insulin icodec - EMEA-002988-PIP01-21

Type 2 diabetes mellitus

Day 30 discussion

Action: For discussion

Endocrinology-Gynaecology-Fertility-Metabolism

3.1.37. Aldafermin - EMEA-003005-PIP01-21

Treatment of non-alcoholic steatohepatitis (NASH)

Day 30 discussion

Action: For discussion

Gastroenterology-Hepatology

3.1.38. [Recombinant adeno-associated viral \(rAAV\) vector expressing the human ornithine transcarbamylase \(hOTC\) gene - EMEA-002983-PIP01-21](#)

Ornithine transcarbamylase deficiency (OTCD)

Day 30 discussion

Action: For discussion

Gastroenterology-Hepatology

3.1.39. [Sirolimus - EMEA-002982-PIP01-21](#)

Ornithine transcarbamylase deficiency (OTCD)

Day 30 discussion

Action: For discussion

Gastroenterology-Hepatology

3.1.40. [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 30 discussion

Action: For discussion

Haematology-Hemostaseology

3.1.41. [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 30 discussion

Action: For discussion

Haematology-Hemostaseology

3.1.42. [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 30 discussion

Action: For discussion

Haematology-Hemostaseology

3.1.43. Efgartigimod alfa - Orphan - EMEA-002597-PIP04-21

argenx BV; Treatment of immune thrombocytopenia

Day 30 discussion

Action: For discussion

Haematology-Hemostaseology

3.1.44. Apremilast - EMEA-000715-PIP06-21

Treatment of coronavirus disease (COVID-19)

Day 30 discussion

Action: For discussion

Immunology-Rheumatology-Transplantation

3.1.45. Leniolisib phosphate - Orphan - EMEA-002989-PIP01-21

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

Day 30 discussion

Action: For discussion

Immunology-Rheumatology-Transplantation

3.1.46. Favipiravir - EMEA-002985-PIP01-21

Treatment of coronavirus disease (COVID-19)

Day 30 discussion

Action: For discussion

Infectious Diseases

3.1.47. Givinostat - Orphan - EMEA-000551-PIP04-21

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

Day 30 discussion

Action: For discussion

Neurology

3.1.48. Immunoglobulin G1 anti-SORT1 human monoclonal antibody - EMEA-002997-PIP01-21

Treatment of dementia

Day 30 discussion

Action: For discussion

Neurology

3.1.49. EMEA-002993-PIP01-21

Treatment of narcolepsy

Day 30 discussion

Action: For discussion

Neurology

3.1.50. EMEA-002635-PIP02-21

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

Day 30 discussion

Action: For discussion

Oncology

3.1.51. Alectinib - EMEA-002431-PIP02-21

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

Day 30 discussion

Action: For discussion

Oncology

3.1.52. Autologous T cells transduced with lentiviral vector containing a tandem chimeric antigen receptor directed against CD20 and CD19 - Orphan - EMEA-003009-PIP01-21

Miltenyi Biomedicine GmbH; Diffuse large B cell lymphoma / Mature aggressive B cell non-Hodgkin lymphoma

Day 30 discussion

Action: For discussion

Oncology

3.1.53. Cetrelimab - EMEA-003006-PIP01-21

Treatment of urothelial carcinoma

Day 30 discussion

Action: For discussion

Oncology

3.1.54. Datopotamab deruxtecan - EMEA-002976-PIP02-21

Treatment of breast cancer

Day 30 discussion

Action: For discussion

Oncology

3.1.55. Gemcitabine HCl - EMEA-003007-PIP01-21

Treatment of urothelial carcinoma

Day 30 discussion

Action: For discussion

Oncology

3.1.56. Autologous CD34+ hematopoietic stem and progenitor cells (HSPCs) genetically modified with the lentiviral vector IDUA LV, encoding for the human α -L-iduronidase (IDUA) cDNA - Orphan - EMEA-003001-PIP01-21

Orchard Therapeutics Netherlands B.V.; Treatment of Mucopolysaccharidosis type I, Hurler syndrome (MPS-IH)

Day 30 discussion

Action: For discussion

Other

3.1.57. Gabapentin - EMEA-002994-PIP01-21

Treatment of postherpetic neuralgia

Day 30 discussion

Action: For discussion

Pain / Neurology

3.1.58. Sivopixant - EMEA-003010-PIP01-21

Unexplained or refractory chronic cough

Day 30 discussion

Action: For discussion

Pneumology - Allergology

3.1.59. EMEA-002990-PIP01-21

Major depressive disorder

Day 30 discussion

Action: For discussion

Psychiatry

3.1.60. Ralmitaront - EMEA-003003-PIP01-21

Treatment of schizophrenia

Day 30 discussion

Action: For discussion

Psychiatry

3.1.61. EMEA-003002-PIP01-21

Treatment of proteinuric chronic kidney disease

Day 30 discussion

Action: For discussion

Uro-nephrology

3.1.62. Escherichia coli vaccine - EMEA-002996-PIP01-21

Prevention of *E.coli* infections

Day 30 discussion

Action: For discussion

Vaccines

3.1.63. Live, attenuated, dengue virus, serotype 4 (DENV4) / Live, attenuated, dengue virus, serotype 3 (DENV3) / Live, attenuated, chimeric dengue virus, serotype 2 (DENV2) / Live, attenuated, dengue virus, serotype 1 (DENV1) - EMEA-002999-PIP01-21

Prevention of dengue disease

Day 30 discussion

Action: For discussion

Vaccines

3.1.64. Recombinant SARS-CoV-2 spike (S)-protein virus-like particle - EMEA-003008-PIP01-21

Prevention of COVID-19 (coronavirus disease 2019)

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. Enalapril maleate - EMEA-C-001706-PIP01-14-M03

Proveca Pharma Limited; Treatment of heart failure

Day 30 discussion

Action: For discussion

Cardiovascular Diseases

3.2.2. Exenatide - EMEA-C-000689-PIP01-09-M11

AstraZeneca AB; Treatment of type 2 diabetes mellitus

Day 30 discussion

Action: For discussion

Endocrinology-Gynaecology-Fertility-Metabolism

3.2.3. Maralixibat Chloride - EMEA-C1-001475-PIP02-13-M01

Mirum Pharmaceuticals; Treatment of Alagille syndrome

Day 30 discussion

Action: For discussion

Gastroenterology-Hepatology

3.2.4. Cobimetinib - EMEA-C-001425-PIP01-13-M05

Roche Registration GmbH; Treatment of all conditions included in the category of malignant neoplasms (except haematopoietic and lymphoid tissue) with Ras, Raf or MEK pathway activation

Day 30 discussion

Action: For discussion

Oncology

3.2.5. Tezepelumab - EMEA-C1-001613-PIP01-14-M04

AstraZeneca AB; Treatment of asthma

Day 30 discussion

Action: For discussion

Pneumology - Allergology

3.3. Discussions on Modification of an Agreed Paediatric Investigation Plan

3.3.1. Clevidipine - EMEA-000282-PIP01-08-M03

Chiesi Farmaceutici S.p.A.; Treatment of hypertensive disease

Day 30 discussion

Action: For discussion

Cardiovascular Diseases

3.3.2. Sacubitril/valsartan - EMEA-000316-PIP02-11-M05

Novartis Europharm Ltd.; Heart failure

Day 30 discussion

Action: For discussion

Cardiovascular Diseases

3.3.3. Baricitinib - EMEA-001220-PIP03-16-M02

Eli Lilly and Company Limited; Treatment of atopic dermatitis

Day 30 discussion

Action: For discussion

Dermatology

3.3.4. Dupilumab - EMEA-001501-PIP01-13-M07

Regeneron Pharmaceuticals, Inc; Atopic dermatitis

Day 30 discussion

Action: For discussion

Dermatology

3.3.5. Lebrikizumab - EMEA-002536-PIP01-18-M01

Eli Lilly and Company Limited; Treatment of atopic dermatitis

Day 30 discussion

Action: For discussion

Dermatology

3.3.6. Ligelizumab - EMEA-001811-PIP02-15-M04

Novartis Europharm Limited; Treatment of chronic spontaneous urticaria

Day 30 discussion

Action: For discussion

Dermatology

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

LEO Pharma A/S; Treatment of atopic dermatitis

Day 30 discussion

Action: For discussion

Dermatology

3.3.8. Liraglutide - EMEA-000128-PIP02-09-M04

Novo Nordisk A/S; Obesity

Day 30 discussion

Action: For discussion

Endocrinology-Gynaecology-Fertility-Metabolism

3.3.9. Ferric maltol - EMEA-001195-PIP01-11-M05

Norgine BV; Treatment of iron deficiency

Day 30 discussion

Action: For discussion

Haematology-Hemostaseology

3.3.10. Giroctocogene fitelparvovec - Orphan - EMEA-002724-PIP01-19-M01

Pfizer Europe MA EEIG; Treatment of haemophilia A

Day 30 discussion

Action: For discussion

Haematology-Hemostaseology

3.3.11. RURIOCTOCOG ALFA PEGOL - EMEA-001296-PIP01-12-M04

Baxalta Innovations GmbH; Haemophilia A (congenital factor VIII deficiency)

Day 30 discussion

Action: For discussion

Haematology-Hemostaseology

3.3.12. Tocilizumab - EMEA-000309-PIP04-17-M03

Roche Registration GmbH; Treatment of cytokine release syndrome

Day 30 discussion

Action: For discussion

Immunology-Rheumatology-Transplantation

3.3.13. Cenobamate - EMEA-002563-PIP02-19-M01

A.C.R.A.F. SpA; Treatment of epilepsy

Day 30 discussion

Action: For discussion

Neurology

3.3.14. Ocrelizumab - EMEA-000310-PIP03-10-M05

Roche Registration GmbH; Treatment of multiple sclerosis

Day 30 discussion

Action: For discussion

Neurology

3.3.15. Phenobarbital - EMEA-002532-PIP01-18-M01

Proveca Pharma Limited; Treatment of epilepsy

Day 30 discussion

Action: For discussion

Neurology

3.3.16. Temelimab - EMEA-002127-PIP01-17-M01

GeNeuro SA; Treatment of multiple sclerosis

Day 30 discussion

Action: For discussion

Neurology

3.3.17. Cabozantinib - Orphan - EMEA-001143-PIP01-11-M03

Ipsen Pharma; Treatment of malignant solid tumours

Day 30 discussion

Action: For discussion

Oncology

3.3.18. Cemiplimab - EMEA-002007-PIP02-17-M01

Regeneron Ireland DAC; Treatment of all conditions included in the category of malignant neoplasms (except haematopoietic and lymphoid tissue)

Day 30 discussion

Action: For discussion

Oncology

3.3.19. Ibrutinib - Orphan - EMEA-001397-PIP03-14-M06

Janssen-Cilag International N.V.; Treatment of mature B-cell neoplasm

Day 30 discussion

Action: For discussion

Oncology

3.3.20. Quizartinib - Orphan - EMEA-001821-PIP01-15-M05

Daiichi Sankyo Europe GmbH; Treatment of acute myeloid leukaemia

Day 30 discussion

Action: For discussion

Oncology

3.3.21. Sirolimus - Orphan - EMEA-001416-PIP01-12-M03

Santen Incorporated; Treatment of non-infectious uveitis affecting the posterior segment of the eye

Day 30 discussion

Action: For discussion

Ophthalmology

3.3.22. [Andexanet alfa - EMEA-001902-PIP01-15-M05](#)

Alexion Europe SAS; Treatment of factor Xa inhibitor associated haemorrhage / Prevention of factor Xa inhibitor associated haemorrhage

Day 30 discussion

Action: For discussion

Other

3.3.23. [In vitro expanded autologous human articular chondrocytes - EMEA-002217-PIP01-17-M02](#)

TETEC Tissue Engineering Technologies AG; Treatment of cartilage disorders

Day 30 discussion

Action: For discussion

Other

3.3.24. [Selexipag - EMEA-000997-PIP01-10-M05](#)

Janssen-Cilag International NV; Treatment of pulmonary arterial hypertension

Day 30 discussion

Action: For discussion

Other

3.3.25. [Xylitol / Procaine hydrochloride / Magnesium sulphate heptahydrate / Potassium chloride - EMEA-001171-PIP01-11-M02](#)

MIT Gesundheit GmbH; Cardioplegia

Day 30 discussion

Action: For discussion

Other

3.3.26. [Lactobacillus reuteri - Orphan - EMEA-001895-PIP01-15-M01](#)

Infant Bacterial Therapeutics AB; Prevention of necrotising enterocolitis

Day 30 discussion

Action: For discussion

Other / Neonatology - Paediatric Intensive Care / Gastroenterology-Hepatology

3.3.27. Fasinumab - EMEA-002059-PIP02-19-M01

Regeneron Ireland D.A.C.; Chronic musculoskeletal pain / Chronic pain / Chronic non-musculoskeletal pain

Day 30 discussion

Action: For discussion

Pain

3.3.28. Seltorexant - EMEA-002746-PIP01-20-M01

Janssen-Cilag International NV; Major depressive disorder (MDD)

Day 30 discussion

Action: For discussion

Psychiatry

3.3.29. Vortioxetine - EMEA-000455-PIP02-10-M08

H. Lundbeck A/S; Major depressive disorder

Day 30 discussion

Action: For discussion

Psychiatry

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

Bayer AG; Chronic kidney disease

Day 30 discussion

Action: For discussion

Uro-nephrology

3.3.31. Ad26.ZEBOV - EMEA-002307-PIP01-17-M02

Janssen Cilag International NV; Prevention of Ebola virus disease

Day 30 discussion

Action: For discussion

Vaccines

3.3.32. Hepatitis B (rDNA) surface antigen adjuvanted - EMEA-001127-PIP02-11-M01

Dynavax GmbH; Prevention of hepatitis B virus infection

Day 30 discussion

Action: For discussion

Vaccines

3.3.33. MVA-BN-Filo - EMEA-002308-PIP01-17-M02

Janssen Cilag International NV; Prevention of Ebola virus disease

Day 30 discussion

Action: For discussion

Vaccines

3.3.34. Neisseria meningitidis serogroup W polysaccharide conjugated to tetanus toxoid / Neisseria meningitidis serogroup Y polysaccharide conjugated to tetanus toxoid / Neisseria meningitidis serogroup C polysaccharide conjugated to tetanus toxoid / Neisseria meningitidis serogroup A polysaccharide conjugated to tetanus toxoid - EMEA-001930-PIP01-16-M03

Sanofi Pasteur; Prevention of invasive meningococcal disease

Day 30 discussion

Action: For discussion

Vaccines

3.3.35. Nirsevimab (MEDI8897) - EMEA-001784-PIP01-15-M03

AstraZeneca AB; Prevention of respiratory syncytial viral infections

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 25 May 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

No items

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

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

7.1.1. Tisagenlecleucel - EMEA-001654-PIP02-17-M01

Novartis Europharm Limited; Treatment of mature B-cell neoplasms

Proposed indications: Treatment of adult patients with r/r follicular lymphoma after two lines of therapies and treatment of adult patients with relapse or refractory aggressive B-cell non-Hodgkin lymphoma after one line of systemic therapy

Action: For adoption

Oncology

7.1.2. Somapacitan - EMEA-001469-PIP02-17

Novo Nordisk A/S; Treatment of short stature

The applicant wishes to receive PDCO's feedback that their newly planned indications "Treatment of Short Stature associated with Noonan Syndrome (NS) / Treatment of Short Stature associated with Turner Syndrome (TS) / Treatment of Idiopathic short stature (ISS)," are covered by the agreed full Waiver with the condition "Treatment of short stature" listed in the Agency Decision.

Action: For discussion

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

No items

9.2. Coordination with EMA Scientific Committees or CMDh-v

9.2.1. Committee for Medicinal Products for Human Use (CHMP)

No items

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)

Action: For information

9.5. Cooperation with International Regulators

No items

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

No items

9.7. PDCO work plan

No items

9.8. Planning and reporting

No items

10. Any other business

10.1. COVID -19 update

Action: For information

10.2. Marketing authorisation applications 3-year forecast report

Action: For information

10.3. Introduction by the Research and Innovation Workstream

Action: For information

11. Breakout sessions

11.1. Paediatric oncology

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

11.2. Neonatology

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/