



EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

18 February 2021
EMA/PDCO/67461/2021
Human Medicines Division

Paediatric Committee (PDCO)

Agenda for the meeting on 23-26 February 2021

Chair: Koenraad Norga – Vice-Chair: Sabine Scherer

23 February 2021, 14:00- 19:00

24 February 2021, 08:30- 19:00

25 February 2021, 08:30- 19:00

26 February 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 23-26 February 2021. See February 2021 PDCO minutes (to be published post March 2021 PDCO meeting).

1.2. Adoption of agenda

PDCO agenda for 23-26 February 2021

1.3. Adoption of the minutes

PDCO minutes for 26-29 January 2021

2. Opinions

Disclosure of some information related to this section cannot be released at the present time as it is deemed to contain commercially confidential information.

2.1. Opinions on Products

2.1.1. Dupilumab - EMEA-001501-PIP07-20

Treatment of chronic spontaneous urticaria

Day 120 opinion

Action: For adoption

Dermatology

2.1.2. Ritlecitinib - EMEA-002451-PIP01-18

Alopecia areata

Day 120 opinion

Action: For adoption

Dermatology

2.1.3. Hydroxypropyl- β -cyclodextrin - Orphan - EMEA-002839-PIP01-20

Cyclo Therapeutics Inc; Niemann Pick disease type C / Treatment of Niemann Pick type C1 disease in children, adolescents and adults

Day 120 opinion

Action: For adoption

Endocrinology-Gynaecology-Fertility-Metabolism

2.1.4. Pioglitazone hydrochloride / Spironolactone / Metformin hydrochloride - EMEA-002187-PIP01-17

Treatment of polycystic ovary syndrome (PCOS) / Treatment of adolescent polycystic ovary syndrome (PCOS) in post-menarche adolescents <18 yrs and young adult women \geq 18 yrs and <24.0 yrs

Day 120 opinion

Action: For adoption

Endocrinology-Gynaecology-Fertility-Metabolism

2.1.5. Risankizumab - EMEA-001776-PIP05-20

Hidradenitis suppurativa

Day 120 opinion

Action: For adoption

Immunology-Rheumatology-Transplantation / Dermatology

2.1.6. Adeno-associated virus, serotype 9 (AAV9)-based non-replicating, self-complementary recombinant vector containing an expression cassette for the human ASPA transgene (scAAV9-CB6-hASPAopt) - Orphan - EMEA-002779-PIP01-20

Aspa Therapeutics, Inc.; Treatment of Canavan disease in patients from birth to less than 18 years of age

Day 120 opinion

Action: For adoption

Neurology

2.1.7. 1-[[[4-(4-fluoro-2-methyl-1H-indol-5-yloxy)-6-methoxyquinolin-7-yl]oxy]methyl]cyclopropanamine-dihydrochloride - Orphan - EMEA-002486-PIP03-20

Advenchen Laboratories, LLC.; Treatment of soft tissue sarcomas / Treatment of alveolar soft part sarcoma / Treatment of synovial sarcoma / Treatment of rhabdomyosarcoma

Day 120 opinion

Action: For adoption

Oncology

2.1.8. Surufatinib - EMEA-002750-PIP01-19

Treatment of all conditions included in the category of malignant neoplasms (except central nervous system tumours and myeloid neoplasms)

Day 120 opinion

Action: For adoption, Oral explanation to be held on Wednesday, 24 February 2021 at 17:00

Oncology

2.1.9. EMEA-002747-PIP01-20

Treatment of myasthenia gravis

Day 120 opinion

Action: For adoption

Other / Neurology

2.1.10. EMEA-002656-PIP01-19

Chikungunya disease

Day 120 opinion

Action: For adoption

Vaccines

2.1.11. Fluoride 18-labelled Prostate-Specific Membrane Antigen-1007 ([¹⁸F]PSMA-1007) - EMEA-002918-PIP01-20

Diagnosis of prostate cancer

Day 60 opinion

Action: For adoption

Diagnostic / Oncology

2.1.12. Sutimlimab - Orphan - EMEA-002542-PIP03-20

Genzyme Europe B.V.; Treatment of cold agglutinin disease

Day 60 opinion

Action: For adoption

Haematology-Hemostaseology

2.1.13. Anti-IL-7Ra monoclonal antibody - EMEA-002930-PIP01-20

Treatment of Sjögren's syndrome

Day 60 opinion

Action: For adoption

Immunology-Rheumatology-Transplantation

2.1.14. Anti-alpha-synuclein human monoclonal antibody - EMEA-002936-PIP01-20

Multiple System Atrophy/ Parkinson's disease

Day 60 opinion

Action: For adoption

Neurology

2.1.15. Oxygen / Argon - EMEA-002921-PIP01-20

Treatment of acute ischaemic stroke

Day 60 opinion

Action: For adoption

Neurology

2.1.16. Tavapadon - EMEA-002920-PIP01-20

Treatment of Parkinson's disease

Day 60 opinion

Action: For adoption

Neurology

2.1.17. 2-((4S)-6-(4-chlorophenyl)-1-methyl-4H-benzo[C]isoxazolo[4,5-e]azepin-4-yl) acetamide monohydrate - Orphan - EMEA-002923-PIP01-20

Constellation Pharmaceuticals Inc.; Treatment of myelofibrosis

Day 60 opinion

Action: For adoption

Oncology

2.1.18. Encequidar - Orphan - EMEA-002913-PIP01-20

Athenex Inc.; Breast cancer/ Soft tissue sarcoma / Breast cancer/ Angiosarcoma

Day 60 opinion

Action: For adoption

Oncology

2.1.19. Ensartinib - EMEA-002937-PIP01-20

Treatment of non-small-cell lung cancer

Day 60 opinion

Action: For adoption

Oncology

2.1.20. Humanised recombinant IgG4, Anti-PD-1 monoclonal antibody - EMEA-002939-PIP01-20

Hepatocellular carcinoma

Day 60 opinion

Action: For adoption

Oncology

2.1.21. Nadofaragene firadenovec - EMEA-002376-PIP02-20

Treatment of malignant bladder neoplasms

Day 60 opinion

Action: For adoption

Oncology

2.1.22. Paclitaxel - Orphan - EMEA-002894-PIP01-20

Athenex Inc.; Breast cancer/ Soft tissue sarcoma / Breast cancer/ Angiosarcoma

Day 60 opinion

Action: For adoption

Oncology

2.1.23. Sabatolimab - EMEA-002931-PIP01-20

Treatment of myelodysplastic syndromes

Day 60 opinion

Action: For adoption

Oncology

2.1.24. Sintilimab - EMEA-002919-PIP01-20

Lung malignant neoplasm

Day 60 opinion

Action: For adoption

Oncology

2.1.25. Talazoparib - EMEA-002066-PIP02-20

Treatment of prostate malignant neoplasms/ Treatment of breast malignant neoplasms

Day 60 opinion

Action: For adoption

Oncology

2.2. Opinions on Compliance Check

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

2.2.1. Sofosbuvir / Velpatasvir - EMEA-C-001646-PIP01-14-M02

Gilead Sciences Ireland UC; Treatment of chronic hepatitis C

Day 60 opinion

Action: For adoption

Infectious Diseases

2.2.2. Bilastine - EMEA-C1-000347-PIP02-16-M02

FAES FARMA, S.A.; Treatment of allergic conjunctivitis

Day 60 letter

Action: For adoption

Ophthalmology / Pneumology – Allergology

2.2.3. Cerliponase alfa - EMEA-C-001362-PIP01-12-M03

BioMarin International Limited; Treatment of Neuronal Ceroid Lipofuscinosis Type 2 (NCL2)

Day 60 opinion

Action: For adoption

Other / Neurology

2.3. Opinions on Modification of an Agreed Paediatric Investigation Plan

2.3.1. [Ambrisentan - Orphan - EMEA-000434-PIP01-08-M08](#)

Glaxo Group Limited; Pulmonary Arterial Hypertension / Treatment of Pulmonary Arterial Hypertension

Day 60 opinion

Action: For adoption

Cardiovascular Diseases

2.3.2. [Azilsartan medoxomil - EMEA-000237-PIP01-08-M09](#)

Takeda Development Centre Europe Ltd; Treatment of hypertension / Essential (primary) hypertension / Secondary hypertension

Day 60 opinion

Action: For adoption

Cardiovascular Diseases

2.3.3. [Landiolol Hydrochloride - EMEA-001150-PIP02-13-M04](#)

AOP Orphan Pharmaceuticals AG; Treatment of supraventricular arrhythmias

Day 60 opinion

Action: For adoption

Cardiovascular Diseases

2.3.4. [Bimekizumab - EMEA-002189-PIP01-17-M02](#)

UCB Biopharma SRL; Treatment of psoriasis / Treatment of moderate to severe chronic plaque psoriasis in children from the age of 6 years and older

Day 60 opinion

Action: For adoption

Dermatology

2.3.5. [Gadopiclenol - EMEA-001949-PIP01-16-M04](#)

Guerbet; Diagnostic / MRI in brain (intracranial), spine and associated tissues to detect and visualize areas with disruption of the blood brain barrier (BBB) and/or abnormal vascularity

Day 60 opinion

Action: For adoption

Diagnostic

2.3.6. Gadopiclenol - EMEA-001949-PIP02-18-M01

Guerbet; Diagnostic / Detection and visualization of disorders or lesions with suspected abnormal vascularity in various body regions

Day 60 opinion

Action: For adoption

Diagnostic

2.3.7. Ethinyl estradiol / Dienogest - EMEA-002229-PIP01-17-M02

Chemo Research; Contraception/ Treatment of women with polycystic ovary syndrome (PCOS) who are not seeking pregnancy / Oral contraception

Day 60 opinion

Action: For adoption

Endocrinology-Gynaecology-Fertility-Metabolism

2.3.8. Ladarixin - EMEA-002642-PIP01-19-M03

Dompé farmaceutici S.p.A; Treatment of type 1 diabetes / Treatment of new-onset type 1 diabetes mellitus

Day 60 opinion

Action: For adoption

Endocrinology-Gynaecology-Fertility-Metabolism

2.3.9. Volanesorsen - Orphan - EMEA-001915-PIP01-15-M03

Akcea Therapeutics; Familial Chylomicronemia Syndrome

Day 60 opinion

Action: For adoption

Endocrinology-Gynaecology-Fertility-Metabolism

2.3.10. Golimumab - EMEA-000265-PIP02-11-M03

Janssen Biologics B.V.; Ulcerative Colitis / Treatment of ulcerative colitis

Day 60 opinion

Action: For adoption

Gastroenterology-Hepatology

2.3.11. Maralixibat - Orphan - EMEA-001475-PIP02-13-M01

Mirum Pharmaceuticals; Treatment of Alagille Syndrome (ALGS)

Day 60 opinion

Action: For adoption

Gastroenterology-Hepatology

2.3.12. Potassium chloride / Sodium chloride / Citric acid, anhydrous / Sodium citrate / Simeticone / Sodium sulphate, anhydrous / Macrogol 4000 - EMEA-001356-PIP02-12-M04

Alfasigma S.p.A.; Any clinical procedures requiring a clean bowel e.g. bowel endoscopy or radiology

Day 60 opinion

Action: For adoption

Gastroenterology-Hepatology

2.3.13. Valoctocogene roxaparvovec - Orphan - EMEA-002427-PIP01-18-M01

BioMarin International Limited; Treatment of Haemophilia A

Day 60 opinion

Action: For adoption

Haematology-Hemostaseology

2.3.14. Upadacitinib - EMEA-001741-PIP01-14-M04

AbbVie Ltd; Treatment of Chronic Idiopathic Arthritis / Treatment of Juvenile Idiopathic Arthritis

Day 60 opinion

Action: For adoption

Immunology-Rheumatology-Transplantation

2.3.15. Upadacitinib - EMEA-001741-PIP04-17-M02

AbbVie Ltd; Treatment of atopic dermatitis

Day 60 opinion

Action: For adoption

Immunology-Rheumatology-Transplantation / Dermatology

2.3.16. Oritavancin (diphosphate) - EMEA-001270-PIP01-12-M03

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

Day 60 opinion

Action: For adoption

Infectious Diseases

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

Gilead Sciences International Limited; Treatment of chronic viral hepatitis B / Treatment of chronic hepatitis B in paediatric patients from 2 years of age with compensated liver disease

Day 60 opinion

Action: For adoption

Infectious Diseases

2.3.18. Dermatophagoides farinae / Dermatophagoides pteronyssinus - EMEA-001258-PIP01-11-M07

ALK-Abelló A/S; Treatment of allergic rhinitis / Treatment of asthma

Day 60 opinion

Action: For adoption

Pneumology - Allergology

2.3.19. Mometasone (furoate) / Indacaterol (acetate) - EMEA-001217-PIP01-11-M07

Novartis Europharm Limited; Treatment of asthma

Day 60 opinion

Action: For adoption

Pneumology - Allergology

2.3.20. Peanut flour - EMEA-001734-PIP01-14-M05

Aimmune Therapeutics Inc; Peanut Allergy / Peanut oral immunotherapy for the reduction in clinical reactivity to accidental exposure in peanut children and adults

Day 60 opinion

Action: For adoption

Pneumology - Allergology

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

For the following partial compliance checks, no need to refer them to PDCO Committee for discussion, were identified by the PME coordinator and PDCO Rapporteur. The PDCO has been informed in writing.

2.7.1. Dupilumab - EMEA-C2-001501-PIP02-13-M06

sanofi-aventis groupe; Treatment of asthma

Day 1 letter

Action: For information

Pneumology – Allergology

2.7.2. Pretomanid - EMEA-C1-002115-PIP01-17-M02

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

Day 1 letter

Action: For information

Infectious Diseases

3. Discussion of applications

Disclosure of some information related to this section cannot be released at the present time as it is deemed to contain commercially confidential information.

3.1. Discussions on Products D90-D60-D30

3.1.1. RAAV8-hUGT1A1 - Orphan - EMEA-002021-PIP01-16

GENETHON; Treatment of Crigler-Najjar syndrome / Treatment of Severe Crigler-Najjar syndrome requiring phototherapy

Day 90 discussion

Action: For discussion

Endocrinology-Gynaecology-Fertility-Metabolism

3.1.2. Vedolizumab - EMEA-000645-PIP04-20

Active pouchitis (in patients who underwent proctocolectomy and ileal-pouch anal anastomosis for ulcerative colitis, amilial adenomatous polyposis, and other underlying conditions for which construction of a pouch was medically indicated) / Active pouchitis (in patients who underwent proctocolectomy and ileal-pouch anal anastomosis for ulcerative colitis) / Adults, Paediatrics

Day 90 discussion

Action: For discussion

Gastroenterology-Hepatology

3.1.3. Pegfilgrastim - EMEA-002671-PIP02-20

Treatment of chemotherapy-induced neutropenia and Prevention of chemotherapy-induced febrile neutropenia / Reduction in the duration of neutropenia and the incidence of febrile neutropenia in paediatric patients treated with cytotoxic chemotherapy for malignancy (with the exception of chronic myeloid leukaemia and myelodysplastic syndromes)

Day 90 discussion

Action: For discussion

Haematology-Hemostaseology

3.1.4. Human anti-interleukin-15 (IL-15) monoclonal antibody - EMEA-002775-PIP01-20

Non-responsive coeliac disease (NRCD) with symptoms despite 12 months of following a gluten-free diet (GFD)

Day 90 discussion

Action: For discussion

Immunology-Rheumatology-Transplantation / Gastroenterology-Hepatology

3.1.5. Edaravone - Orphan - EMEA-002785-PIP01-20

Treeway B.V.; Treatment of amyotrophic lateral sclerosis

Day 90 discussion

Action: For discussion

Neurology

3.1.6. EMEA-002795-PIP01-20

Prevention of lower respiratory tract disease caused by respiratory syncytial virus via maternal immunisation / Prevention of RSV-associated medically attended lower respiratory tract illness (MA-LRTI) and/or RSV associated severe MA-LRTI in neonates and infants by active immunisation of pregnant adolescents

Day 90 discussion

Action: For discussion

Vaccines

3.1.7. Ruxolitinib - EMEA-002618-PIP02-20

Vitiligo

Day 60 discussion

Action: For discussion

Dermatology

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

Psoriasis / Treatment of mild to moderate plaque psoriasis in patients ≥ 2 years to < 18 years

Day 60 discussion

Action: For discussion

Dermatology

3.1.9. Glepaglutide - EMEA-002926-PIP01-20

Short bowel syndrome (SBS)

Day 60 discussion

Action: For discussion

Gastroenterology-Hepatology

3.1.10. (S)-1-(5-(((2,3-dihydro-[1,4]dioxino[2,3-b]pyridin-7-yl)sulfonyl)-3,4,5,6-tetrahydropyrrolo[3,4-c]pyrrol-2(1H)-yl)-3-hydroxy-2-phenylpropan-1-one - Orphan - EMEA-002924-PIP01-20

Forma Therapeutics, Inc.; Sickle Cell Disease

Day 60 discussion

Action: For discussion

Haematology-Hemostaseology

3.1.11. Concizumab - Orphan - EMEA-002326-PIP04-20

Novo Nordisk A/S; Treatment of congenital haemophilia B / Treatment of congenital haemophilia A

Day 60 discussion

Action: For discussion

Haematology-Hemostaseology

3.1.12. EMEA-002927-PIP01-20

Chronic idiopathic arthritis / Treatment of juvenile idiopathic arthritis

Day 60 discussion

Action: For discussion

Immunology-Rheumatology-Transplantation

3.1.13. EMEA-002928-PIP01-20

Treatment of plaque psoriasis / Treatment of moderate-to-severe plaque psoriasis in children and adolescents age 6 to <18 years who are candidates for systemic therapies

Day 60 discussion

Action: For discussion

Immunology-Rheumatology-Transplantation / Dermatology / Gastroenterology-Hepatology

3.1.14. Islatravir - EMEA-002938-PIP01-20

Prevention of human immunodeficiency virus (HIV-1) infection

Day 60 discussion

Action: For discussion

Infectious Diseases

3.1.15. Branaplam - EMEA-002204-PIP02-20

Treatment of Huntington's disease

Day 60 discussion

Action: For discussion

Neurology

3.1.16. Crisantaspase - EMEA-002934-PIP01-20

Treatment of acute lymphoblastic leukaemia / lymphoma / Acute lymphoblastic leukaemia

(ALL)/lymphoblastic lymphoma (LBL)

Day 60 discussion

Action: For discussion

Oncology

3.1.17. [Loncastuximab tesirine - EMEA-002665-PIP02-20](#)

Treatment of B-cell non-Hodgkin Lymphoma

Day 60 discussion

Action: For discussion

Oncology

3.1.18. [Sepofarsen - Orphan - EMEA-002717-PIP02-20](#)

ProQR Therapeutics; Treatment of inherited retinal disorders / Treatment of Leber congenital amaurosis due to the C2991 +1655A>G mutation in the CEP290 gene (p.Cys998X)

Day 60 discussion

Action: For discussion

Ophthalmology

3.1.19. [Ligelizumab - EMEA-001811-PIP03-20](#)

Treatment of food allergy

Day 60 discussion

Action: For discussion

Pneumology - Allergology

3.1.20. [Sodium chloride solution 4.2% \(w/v\) / 3,5-diamino-6-chloro-N-\(N-\(4-\(4-\(2-\(hexyl\(\(2S,3R,4R,5R\)-2,3,4,5,6-pentahydroxyhexyl\)amino\)ethoxy\)phenyl\)butyl\)-carbamimidoyl\)pyrazine-2-carboxamide - Orphan - EMEA-002935-PIP01-20](#)

Parion Sciences, Inc.; Primary Ciliary Dyskinesia (PCD)

Day 60 discussion

Action: For discussion

Pneumology - Allergology

3.1.21. [Recombinant protein derived from the SARS CoV2 prefusion Spike delta TM protein - EMEA-002915-PIP01-20](#)

Prevention of Covid-19 / Prevention of Covid-19 disease / Prevention of Covid-19 infection

Day 60 discussion

Action: For discussion

Vaccines

3.1.22. [EMEA-002948-PIP01-20](#)

Treatment of dilated cardiomyopathy

Day 30 discussion

Action: For discussion

Cardiovascular Diseases

3.1.23. [EMEA-002735-PIP03-20](#)

Heart transplantation / Preservation of hearts prior to heart transplantation

Day 30 discussion

Action: For discussion

Cardiovascular Diseases

3.1.24. [EMEA-002944-PIP01-20](#)

Treatment of Type 2 Diabetes Mellitus

Day 30 discussion

Action: For discussion

Endocrinology-Gynaecology-Fertility-Metabolism

3.1.25. [Peptide derivative of glucagon-like-peptide 1 and glucagon with fatty acid side chain - EMEA-002942-PIP01-20](#)

Treatment of NASH/NAFLD with fibrosis

Day 30 discussion

Action: For discussion

Gastroenterology-Hepatology

3.1.26. [Peptide derivative of glucagon-like-peptide 1 and glucagon with fatty acid side chain - EMEA-002942-PIP02-20](#)

Treatment of obesity

Day 30 discussion

Action: For discussion

Gastroenterology-Hepatology

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

Catalyst Biosciences, Inc.; Treatment of haemophilia A / Treatment of bleeding episodes in patients with congenital haemophilia A with inhibitors to Factor VIII

Day 30 discussion

Action: For discussion

Haematology-Hemostaseology

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

Catalyst Biosciences, Inc.; Treatment of haemophilia B / Treatment of bleeding episodes in patients with congenital HB with inhibitors to Factor IX

Day 30 discussion

Action: For discussion

Haematology-Hemostaseology

3.1.29. Anti-CD40L humanized monoclonal antibody - EMEA-002945-PIP01-20

Treatment of Sjogren's Syndrome

Day 30 discussion

Action: For discussion

Immunology-Rheumatology-Transplantation

3.1.30. Baricitinib - EMEA-001220-PIP08-20

Treatment of alopecia areata

Day 30 discussion

Action: For discussion

Immunology-Rheumatology-Transplantation

3.1.31. Exebacase - EMEA-002947-PIP01-20

Treatment of *Staphylococcus aureus* bloodstream infections (bacteraemia)

Day 30 discussion

Action: For discussion

Infectious Diseases

3.1.32. Reparixin - Orphan - EMEA-001693-PIP02-20

Dompé farmaceutici S.p.A.; Treatment of COVID-19 pneumonia / Treatment of severe COVID-19 pneumonia

Day 30 discussion

Action: For discussion

Infectious Diseases

3.1.33. Terbinafine - EMEA-001259-PIP03-20

Treatment of onychomycosis / Treatment of distal subungual onychomycosis

Day 30 discussion

Action: For discussion

Infectious Diseases

3.1.34. EMEA-002940-PIP01-20

Treatment of coronavirus disease 2019 (COVID-19)

Day 30 discussion

Action: For discussion

Infectious Diseases

3.1.35. EMEA-002943-PIP01-20

Mature B-Cell malignancies

Day 30 discussion

Action: For discussion

Oncology

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

Advanced Accelerator Applications; Gastroenteropancreatic neuroendocrine tumours (GEP-NETs) / Treatment of unresectable or metastatic, progressive, well differentiated (G1 and G2), somatostatin receptor positive gastroenteropancreatic neuroendocrine tumours (GEP-NETs) in adolescent patients (12 years and older)

Day 30 discussion

Action: For discussion

Oncology

3.1.37. Pembrolizumab / quavonlimab - EMEA-002949-PIP01-20

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

Day 30 discussion

Action: For discussion

Oncology

3.1.38. Apitegromab - Orphan - EMEA-002951-PIP01-20

Scholar Rock, Inc.; Spinal muscular atrophy

Day 30 discussion

Action: For discussion

Other / Neurology

3.1.39. EMEA-002946-PIP01-20

Treatment of Major Depressive Disorder (MDD) / Rapid reduction of depressive symptoms, in conjunction with comprehensive standard of care (SoC), in adolescents with MDD who have suicidal ideation with intent

Day 30 discussion

Action: For discussion

Psychiatry

3.1.40. Adjuvant / Severe acute respiratory syndrome coronavirus 2 (SARS-CoV-2) recombinant (r) spike (S) protein nanoparticle vaccine (SARS CoV-2 rS) - EMEA-002941-PIP01-20

Prevention of COVID-19 caused by SARS-CoV-2 infection / Active immunisation for the prevention of mild, moderate, and severe disease caused by SARS-CoV-2 in children 6 months through 17 years of age

Day 30 discussion

Action: For discussion

Vaccines

3.1.41. Regdanvimab - EMEA-002961-PIP01-21

Treatment of Coronavirus disease 2019 (COVID-19)

Day 30 discussion

Action: For discussion

Infectious Diseases

3.1.42. [Bamlanivimab - EMEA-002952-PIP01-21](#)

Treatment of coronavirus disease 2019 (COVID-19)

Day 30 discussion

Action: For discussion

Infectious Diseases

3.1.43. [Etesevimab - EMEA-002966-PIP01-21](#)

Treatment of coronavirus disease 2019 (COVID-19)

Day 30 discussion

Action: For discussion

Infectious Diseases

3.1.44. [EMEA-002964-PIP01-21](#)

Prophylaxis of SARS-CoV-2 infection / Treatment and decreased transmission of coronavirus disease 2019 (COVID-19)

Day 30 discussion

Action: For discussion

Infectious Diseases

3.1.45. [EMEA-002965-PIP01-21](#)

Prophylaxis of SARS-CoV-2 infection / Treatment and decreased transmission of coronavirus disease 2019 (COVID-19)

Day 30 discussion

Action: For discussion

Infectious Diseases

3.2. **Discussions on Compliance Check**

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

3.2.1. [Simoctocog alfa - EMEA-C-001024-PIP01-10-M02](#)

Octapharma Pharmazeutika Produktionsges.m.b.H.; Hereditary Factor VIII deficiency / Haemophilia A

Day 30 discussion

Action: For discussion

3.2.2. Lenvatinib - EMEA-C3-001119-PIP02-12-M07

Eisai GmbH; Treatment of papillary thyroid cancer/ Treatment of follicular thyroid cancer/
Treatment of osteosarcoma

Day 30 discussion

Action: For discussion

Oncology

3.3. Discussions on Modification of an Agreed Paediatric Investigation Plan

3.3.1. Pegcetacoplan - Orphan - EMEA-002600-PIP01-19-M01

Apellis Ireland Limited; Paroxysmal Nocturnal Haemoglobinuria

Day 30 discussion

Action: For discussion

3.3.2. Remimazolam - EMEA-001880-PIP02-19-M02

PAION Deutschland GmbH; Sedation, General anesthesia / Sedation of mechanically
ventilated patients / Sedation for short procedures

Day 30 discussion

Action: For discussion

Anaesthesiology

3.3.3. Macitentan - Orphan - EMEA-001032-PIP01-10-M03

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

Day 30 discussion

Action: For discussion

Cardiovascular Diseases

3.3.4. Ticagrelor - EMEA-000480-PIP01-08-M14

AstraZeneca AB; Thromboembolic events (children), acute coronary syndrome, history of
myocardial infarction / reduction in occurrence of vaso-occlusive crises in paediatric patients
with sickle cell disease

Day 30 discussion

Action: For discussion

Cardiovascular Diseases / Haematology-Hemostaseology

3.3.5. [Spesolimab - EMEA-002475-PIP02-19-M01](#)

Boehringer Ingelheim International GmbH; Prevention of Generalized Pustular Psoriasis / Treatment of Generalized Pustular Psoriasis / treatment of patients with acute or chronic generalized pustular psoriasis (GPP) and for the prevention of flares

Day 30 discussion

Action: For discussion

Dermatology

3.3.6. [Cipaglucosidase alfa - Orphan - EMEA-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

3.3.7. [Pegunigalsidase alfa - Orphan - EMEA-001828-PIP01-15-M02](#)

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

Day 30 discussion

Action: For discussion

Endocrinology-Gynaecology-Fertility-Metabolism

3.3.8. [Avacopan - Orphan - EMEA-002023-PIP01-16-M05](#)

ChemoCentryx Ireland Ltd.; Treatment of anti-neutrophil cytoplasmic auto-antibody (ANCA)-associated vasculitis

Day 30 discussion

Action: For discussion

Immunology-Rheumatology-Transplantation

3.3.9. [Doravirine - EMEA-001676-PIP01-14-M04](#)

Merck Sharp & Dohme (Europe), Inc.; Treatment of human immunodeficiency virus-1 (HIV-1) infection / Antiretroviral therapy, in combination with other antiretroviral agents, for the treatment of HIV-1 infection in children aged from birth to 18 years

Day 30 discussion

Action: For discussion

Infectious Diseases

3.3.10. Rilpivirine / Dolutegravir - EMEA-001750-PIP01-15-M04

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

Day 30 discussion

Action: For discussion

Infectious Diseases

3.3.11. Tenofovir alafenamide / emtricitabine / cobicistat / darunavir - EMEA-001825-PIP01-15-M03

Janssen-Cilag International NV; Treatment of human immunodeficiency virus type-1 (HIV-1) infection / Treatment of HIV-1 infection in paediatric subjects weighing 25 kg or more from 6 years of age

Day 30 discussion

Action: For discussion

Infectious Diseases

3.3.12. Tenofovir disoproxil fumarate / lamivudine / doravirine - EMEA-001695-PIP01-14-M04

Merck Sharp & Dohme (Europe), Inc.; Treatment of human immunodeficiency virus-1 (HIV-1) infection / Antiretroviral combination therapy for the treatment of HIV-1 infection in adults and children aged 6 to 18 years

Day 30 discussion

Action: For discussion

Infectious Diseases

3.3.13. Ataluren - Orphan - EMEA-000115-PIP01-07-M11

PTC Therapeutics International, Limited; Treatment of dystrophinopathy / Treatment of nonsense-mutation dystrophinopathy

Day 30 discussion

Action: For discussion

Neurology

3.3.14. Brivaracetam - Orphan - EMEA-000332-PIP01-08-M16

UCB Pharma S.A.; Treatment of epilepsy with partial onset seizures / Treatment of

paediatric patients with partial onset seizures

Day 30 discussion

Action: For discussion

Neurology

3.3.15. Brivaracetam - Orphan - EMEA-000332-PIP02-17-M02

UCB Pharma S.A.; Treatment of Neonatal Seizures / Treatment of paediatric epilepsy syndromes / Treatment of Neonatal Seizures with adjunctive administration of brivaracetam, Monotherapy in patients 4 to 25 years of age with Childhood Absence Epilepsy (CAE) and Juvenile Absence Epilepsy (JAE)

Day 30 discussion

Action: For discussion

Neurology

3.3.16. Bumetanide - EMEA-001303-PIP01-12-M03

Les Laboratoires Servier; Autism Spectrum Disorder / Treatment of Autism Spectrum Disorder

Day 30 discussion

Action: For discussion

Neurology

3.3.17. Ganaxolone - Orphan - EMEA-002341-PIP01-18-M01

Marinus Pharmaceuticals Inc.; Cyclin-dependent kinase-like 5 (CDKL5) deficiency disorder / Adjunctive treatment of seizures in paediatric patients aged 6 months to < 18 years old with cyclin-dependent kinase-like 5 (CDKL5) deficiency disorder

Day 30 discussion

Action: For discussion

Neurology

3.3.18. Dostarlimab - EMEA-002463-PIP01-18-M01

GlaxoSmithKline (Ireland) Limited; Treatment of all conditions included in the category of malignant neoplasms (except haematopoietic and lymphoid malignancies) / Treatment of paediatric patients from birth to less than 18 years old with neuroblastoma and/or osteosarcoma

Day 30 discussion

Action: For discussion

Oncology

3.3.19. Isatuximab - EMEA-002205-PIP01-17-M02

Sanofi-Aventis Recherche & Développement; Treatment of malignant neoplasms of the haematopoietic and lymphoid tissue / Treatment of relapsed, refractory and newly-diagnosed acute myeloid leukemia in combination with standard treatment (chemotherapy) in paediatric patients from 28 days to less than 18 years of age / Treatment of relapsed, refractory and newly-diagnosed acute lymphoblastic leukemia in combination with standard treatment (chemotherapy) in paediatric patients from 28 days to less than 18 years of age

Day 30 discussion

Action: For discussion

Oncology

3.3.20. Lenvatinib - EMEA-001119-PIP03-19-M01

Eisai GmbH; Treatment of all conditions included in the category of malignant neoplasms except haematopoietic and lymphoid tissue neoplasms, papillary thyroid cancer, follicular thyroid cancer and osteosarcoma / Treatment of paediatric patients from 2 years to less than 18 years old with a relapsed or refractory solid malignant tumour including Ewing sarcoma/peripheral primitive neuroectodermal tumour, rhabdomyosarcoma and high-grade glioma

Day 30 discussion

Action: For discussion

Oncology

3.3.21. Niraparib (as tosylate monohydrate) - Orphan - EMEA-002268-PIP02-18-M01

GlaxoSmithKline (Ireland) Limited; Treatment of all conditions included in the category of malignant neoplasms (except haematopoietic and lymphoid malignancies) / Treatment of paediatric patients from birth to less than 18 years old with neuroblastoma and/or osteosarcoma

Day 30 discussion

Action: For discussion

Oncology

3.3.22. Obinutuzumab - Orphan - EMEA-001207-PIP01-11-M01

Roche Registration GmbH; Treatment of mature B-cell lymphoma / Treatment of acute lymphoblastic leukaemia

Day 30 discussion

Action: For discussion

Oncology

3.3.23. Ruxolitinib phosphate - EMEA-000901-PIP03-16-M02

Novartis Europharm Limited; Acute Graft versus Host Disease / Treatment of acute Graft versus Host Disease (aGvHD) in paediatric patients aged 28 days and above

Day 30 discussion

Action: For discussion

Oncology

3.3.24. Concentrate of proteolytic enzyme enriched in bromelain - Orphan - EMEA-000142-PIP02-09-M11

MediWound Germany GmbH; Treatment of burns of external body surfaces / Removal of eschar in deep partial and/or full thickness burns

Day 30 discussion

Action: For discussion

Other

3.3.25. Meloxicam / Bupivacaine - EMEA-002246-PIP01-17-M02

Heron Therapeutics B.V.; Acute postoperative pain

Day 30 discussion

Action: For discussion

Pain

3.3.26. Birch pollen extract (*Betula verrucosa*) - EMEA-001879-PIP01-15-M03

ALK-Abelló A/S; Treatment of allergic rhinitis / rhino-conjunctivitis / Treatment of tree pollen allergic rhinitis / rhino-conjunctivitis

Day 30 discussion

Action: For discussion

Pneumology - Allergology

3.3.27. Brexpiprazole - EMEA-001185-PIP01-11-M07

Otsuka Pharmaceutical Development & Commercialisation Europe GmbH; Treatment of schizophrenia from 13 to less than 18 years of age

Day 30 discussion

Action: For discussion

Psychiatry

4. Nominations

Disclosure of information related to this section cannot be released at the present time as it is deemed to contain commercially confidential information.

4.1. List of submission of applications with start of procedure 23 February 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

Disclosure of information related to this section cannot be released at the present time as it is deemed to contain commercially confidential information.

6. Discussion on the applicability of class waivers

Disclosure of some information related to this section cannot be released 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. Itepekimab - EMEA-10-2020

Sanofi-aventis Recherche & Développement; All classes of medicinal products for treatment of chronic obstructive pulmonary disease (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) / Add-on maintenance treatment of adult patients with chronic obstructive pulmonary disease who do not currently smoke

Action: For adoption

6.1.2. Avacincaptad pegol - EMEA-11-2020

IVERIC bio, Inc.; All classes of medicinal products for treatment of age-related macular

degeneration and diabetic macular oedema / Treatment of geographic atrophy secondary to dry age-related macular degeneration

Action: For adoption

6.1.3. Semaglutide - EMEA-01-2021

Novo Nordisk; All classes of medicinal products for treatment of Alzheimer's disease / Treatment of mild cognitive impairment and mild dementia, both of the Alzheimer's type

Action : For adoption

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

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

7.1.1. Teplizumab

Provention Bio, Inc.; Type I Diabetes mellitus

Request to confirm condition/indication wording with regards to a possible second PIP for this compound

Action: For discussion

Endocrinology-Gynaecology-Fertility-Metabolism

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. Proposal for criteria to put procedures on the "silent adoption list"

Action: For discussion

9.1.2. Access to IT tools for PDCO members

Action: For information

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

9.3.3. Agenda for the Patients and Consumers Working Party (PCWP)/ Healthcare Professionals Working Party (HCPWP) on 2-3 March 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)

No items

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. Introduction of new Executive Director Emer Cooke

Action: For information

10.3.

Action: For adoption

10.4. C4C multistakeholder meeting on paediatric IBD

Action: For information

10.5.

Action: For information

11. Breakout sessions

11.1. Paediatric oncology

Action: For discussion on Tuesday, 12:00-13:00

11.2. Neonatology

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

11.3. Internal operations

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

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/