



EUROPEAN MEDICINES AGENCY  
SCIENCE MEDICINES HEALTH

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Human Medicines Division

## Paediatric Committee (PDCO)

### Agenda for the meeting on 8-11 December 2020

Chair: Koenraad Norga – Vice-Chair: Sabine Scherer

8 December 2020, 14:00-19:00, Virtual meeting

9 December 2020, 08:30- 19:00, Virtual meeting

10 December 2020, 08:30- 19:00, Virtual meeting

11 December 2020, 08:30- 13:00, Virtual meeting

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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.

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#### 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 8-11 December 2020. See 8-11 December 2020 PDCO minutes (to be published post 26-29 January 2021 PDCO meeting).

### 1.2. Adoption of agenda

PDCO agenda for 8-11 December 2020

### 1.3. Adoption of the minutes

PDCO minutes for 10-13 November 2020

## 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. EMEA-002757-PIP01-19

---

Ulcerative colitis

Day 120 opinion

**Action:** For adoption

Gastroenterology-Hepatology

#### 2.1.2. Obinutuzumab - Orphan - EMEA-001207-PIP02-19

---

Roche Registration GmbH; Systemic Lupus Erythemathosus

Day 120 opinion

**Action:** For adoption

Immunology-Rheumatology-Transplantation

### 2.1.3. Lenacapavir - EMEA-002740-PIP01-19

---

Treatment of human immunodeficiency virus (HIV-1) infection

Day 120 opinion

**Action:** For adoption

Infectious Diseases

### 2.1.4. Retinol (Vitamin A) - Orphan - EMEA-002790-PIP01-20

---

orphanix GmbH; Prevention of Bronchopulmonary Dysplasia (BPD)

Day 120 opinion

**Action:** For adoption

Neonatology - Paediatric Intensive Care

### 2.1.5. Arimoclomol citrate - Orphan - EMEA-001748-PIP03-19

---

Orphazyme A/S; Treatment of Amyotrophic Lateral Sclerosis

Day 120 opinion

**Action:** For adoption

Neurology

### 2.1.6. Autologous tumor-infiltrating lymphocytes - EMEA-002776-PIP01-20

---

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

Day 120 opinion

**Action:** For adoption

Oncology

### 2.1.7. Carfilzomib - Orphan - EMEA-001806-PIP04-19

---

Amgen Europe BV; Treatment of acute lymphoblastic leukemia / Treatment of paediatric patients aged 1 month or older and young adult patients up to 21 years of age with bone marrow relapse of T-cell ALL treated with at least one prior therapy or B-cell ALL treated with prior targeted immune therapy, with or without extramedullary disease

Day 120 opinion

**Action:** For adoption

Oncology / Haematology-Hemostaseology

2.1.8. (S)-6-hydroxy-2,5,7,8-tetramethyl-N-((R)-piperidin-3-yl)chroman-2-carboxamide hydrochloride - Orphan - EMEA-002113-PIP01-16

---

Khondrion BV; Treatment of mitochondrial respiratory chain /oxidative phosphorylation defects

Day 120 opinion

**Action:** For adoption

Other

2.1.9. Dexmedetomidine - EMEA-002758-PIP01-19

---

Treatment of acute agitation in bipolar disorder / Treatment of acute agitation in schizophrenia

Day 120 opinion

**Action:** For adoption

Psychiatry

2.1.10. Esketamine - EMEA-002772-PIP01-20

---

Bipolar depression / Major depressive disorder / Treatment-resistant bipolar depression / Treatment-resistant depression in the course of major depressive disorder

Day 120 opinion

**Action:** For adoption

Psychiatry

2.1.11. Allopurinol / verinurad - EMEA-002754-PIP01-19

---

Chronic kidney disease / Treatment of chronic kidney disease in children and adolescents (6 to <18 years old) with hyperuricaemia and albuminuria

Day 120 opinion

**Action:** For adoption

Uro-nephrology

2.1.12. Sparsentan - Orphan - EMEA-001984-PIP02-20

---

Retrophin Europe Limited; Treatment of focal segmental glomerular sclerosis (FSGS)

Day 120 opinion

**Action:** For adoption

Uro-nephrology

### 2.1.13. Sparsentan - EMEA-001984-PIP03-20

---

Treatment of IgA Nephropathy (IgAN)

Day 120 opinion

**Action:** For adoption

Uro-nephrology

### 2.1.14. Rosuvastatin / acetylsalicylic acid - EMEA-002891-PIP01-20

---

Prevention of cardiovascular events

Day 60 opinion

**Action:** For adoption

Cardiovascular Diseases

### 2.1.15. Heparin - EMEA-002885-PIP01-20

---

Prevention and treatment of thromboembolic events

Day 60 opinion

**Action:** For adoption

Cardiovascular Diseases / Haematology-Hemostaseology

### 2.1.16. Reldesemtiv - Orphan - EMEA-002868-PIP01-20

---

Cytokinetics, Inc.; Amyotrophic lateral sclerosis (ALS)

Day 60 opinion

**Action:** For adoption

Neurology

### 2.1.17. EMEA-002884-PIP01-20

---

Progressive supranuclear palsy

Day 60 opinion

**Action:** For adoption

Neurology

### 2.1.18. EMEA-002745-PIP02-20

---

Treatment of lymphoplasmacytic lymphoma

Day 60 opinion

**Action:** For adoption

Oncology

#### 2.1.19. [Catumaxomab - EMEA-002879-PIP01-20](#)

---

Treatment of malignant ascites

Day 60 opinion

**Action:** For adoption

Oncology

#### 2.1.20. [Lorlatinib - EMEA-002669-PIP02-20](#)

---

Treatment of lung malignant neoplasms

Day 60 opinion

**Action:** For adoption

Oncology

#### 2.1.21. [Ublituximab - Orphan - EMEA-002889-PIP01-20](#)

---

CambPharma Solutions (CY) Ltd; Treatment / Chronic Lymphocytic Leukemia (CLL)

Day 60 opinion

**Action:** For adoption

Oncology

#### 2.1.22. [Umbralisib - EMEA-002890-PIP01-20](#)

---

Treatment chronic lymphocytic leukemia (CLL)

Day 60 opinion

**Action:** For adoption

Oncology

#### 2.1.23. [Paracetamol / nefopam - EMEA-002877-PIP01-20](#)

---

Treatment of acute pain / Symptomatic short-term treatment of moderate to severe somatic acute pain.

Day 60 opinion

**Action:** For adoption

Pain

#### 2.1.24. Eliapixant - EMEA-002882-PIP01-20

---

Treatment of refractory and/or unexplained chronic cough (RUCC)

Day 60 opinion

**Action:** For adoption

Pneumology - Allergology

#### 2.1.25. Alpha1-Proteinase Inhibitor (Human) - EMEA-002888-PIP01-20

---

Treatment of emphysema secondary to alpha 1-proteinase inhibitor deficiency

Day 60 opinion

**Action:** For adoption

Pneumology - Allergology / Haematology-Hemostaseology

#### 2.1.26. Acetylcysteine / Ibuprofen - EMEA-002561-PIP02-20

---

Upper respiratory tract signs and symptoms

Day 60 opinion

**Action:** For adoption

Pneumology - Allergology / Oto-rhino-laryngology

### 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. Riociguat - EMEA-C-000718-PIP01-09-M06

---

Bayer AG; Treatment of pulmonary hypertension

Day 60 opinion

**Action:** For adoption

Cardiovascular Diseases

#### 2.2.2. Idursulfase - EMEA-C-000294-PIP02-12-M01

---

Shire Human Genetic Therapies AB; Treatment of mucopolysaccharidosis II (Hunter syndrome)

Day 60 opinion

**Action:** For adoption

Endocrinology-Gynaecology-Fertility-Metabolism

### 2.2.3. Elbasvir / grazoprevir - EMEA-C-001604-PIP01-13-M03

---

Merck Sharp & Dohme B.V.; Treatment of chronic hepatitis C

Day 60 opinion

**Action:** For adoption

Infectious Diseases

## 2.3. Opinions on Modification of an Agreed Paediatric Investigation Plan

### 2.3.1. Remimazolam (as besylate) - EMEA-001880-PIP02-19-M01

---

PAION Deutschland GmbH; Sedation / General anesthesia / Sedation of mechanically ventilated patients / Procedural sedation

Day 60 opinion

**Action:** For adoption

Anaesthesiology

### 2.3.2. Denosumab - EMEA-000145-PIP02-12-M03

---

Amgen Europe B.V.; Treatment of Osteoporosis / Treatment of osteogenesis imperfecta / Treatment of glucocorticoid induced osteoporosis

Day 60 opinion

**Action:** For adoption

Endocrinology-Gynaecology-Fertility-Metabolism

### 2.3.3. Darvadstrocel - Orphan - EMEA-001561-PIP01-13-M02

---

Takeda Pharma A/S; Perianal fistula

Day 60 opinion

**Action:** For adoption

Gastroenterology-Hepatology

### 2.3.4. Obeticholic Acid - Orphan - EMEA-001304-PIP02-13-M05

---

Intercept Pharma International Ltd.; Primary Biliary Cholangitis (PBC) / Biliary Atresia

Day 60 opinion

**Action:** For adoption

Gastroenterology-Hepatology

### 2.3.5. Tofacitinib - EMEA-000576-PIP03-12-M05

---

Pfizer Europe MA EEIG; Treatment of ulcerative colitis

Day 60 opinion

**Action:** For adoption

Gastroenterology-Hepatology

### 2.3.6. Betibeglogene autotemcel - Orphan - EMEA-001665-PIP01-14-M04

---

bluebird bio (Netherlands) B.V.; Treatment of  $\beta$ -thalassaemia / Treatment of beta-thalassaemia major and severe intermedia

Day 60 opinion

**Action:** For adoption

Haematology-Hemostaseology

### 2.3.7. Luspatercept - Orphan - EMEA-001521-PIP01-13-M05

---

Celgene Europe B.V.; Treatment of beta-thalassaemia / Treatment of anaemia in patients with beta-thalassaemia intermedia and major

Day 60 opinion

**Action:** For adoption

Haematology-Hemostaseology

### 2.3.8. Risankizumab - EMEA-001776-PIP02-17-M01

---

AbbVie Ltd; Chronic idiopathic arthritis

Day 60 opinion

**Action:** For adoption

Immunology-Rheumatology-Transplantation

### 2.3.9. Tofacitinib citrate - EMEA-000576-PIP01-09-M13

---

Pfizer Europe MA EEIG; Treatment of chronic idiopathic arthritis (including rheumatoid arthritis, psoriatic arthritis, ankylosing spondylitis and juvenile idiopathic arthritis) / Juvenile idiopathic arthritis

Day 60 opinion

**Action:** For adoption

Immunology-Rheumatology-Transplantation



2.3.10. [3-\({5-chloro-1-\[3-\(methylsulfonyl\)propyl\]-1Hindol-2-yl}methyl\)-1-\(2,2,2-trifluoroethyl\)-1,3-dihydro-2H-imidazo\[4,5-C\]pyridin-2-one](#) - EMEA-001838-PIP01-15-M03

---

Janssen-Cilag International NV; Treatment of respiratory tract disease caused by human respiratory syncytial virus (RSV) / Treatment of respiratory tract disease caused by human RSV

Day 60 opinion

**Action:** For adoption

Infectious Diseases

2.3.11. [Aztreonam / Avibactam](#) - EMEA-002283-PIP01-17-M01

---

Pfizer Europe MA EEIG; Treatment of infections caused by aerobic gram-negative bacteria / Treatment of infections caused by aerobic gram-negative bacteria in patients with limited therapeutic options

Day 60 opinion

**Action:** For adoption

Infectious Diseases

2.3.12. [Baloxavir marboxil](#) - EMEA-002440-PIP01-18-M01

---

Roche Registration GmbH; Prevention of Influenza / Treatment of Influenza / Treatment of influenza type A/B in otherwise healthy and high risk patients / Prevention (post-exposure prophylaxis) of influenza type A/B / Reduction of transmission of influenza type A/B

Day 60 opinion

**Action:** For adoption

Infectious Diseases

2.3.13. [Ceftazidime / avibactam](#) - EMEA-001313-PIP01-12-M10

---

Pfizer Europe MA EEIG; Treatment of bacterial infections / For the treatment of complicated urinary tract infections / For the treatment of complicated intraabdominal infections / For the treatment of pneumonia / For the treatment of infections due to aerobic Gram-negative organisms

Day 60 opinion

**Action:** For adoption

Infectious Diseases

2.3.14. [Cobicistat](#) - EMEA-000969-PIP01-10-M05

---

Gilead Sciences International Ltd.; Treatment of human immunodeficiency virus type-1

(HIV-1) infection / Treatment of human immunodeficiency virus type-1 (HIV-1) infection -  
pharmacoenhancer for use in combination with antiretroviral agents

Day 60 opinion

**Action:** For adoption

Infectious Diseases

#### 2.3.15. Cobicistat / darunavir - EMEA-001280-PIP01-12-M03

Janssen-Cilag International NV; Treatment of HIV-1 Infection / Treatment of HIV-1 infection  
in pediatric patients from 3 to less than 18 years

Day 60 opinion

**Action:** For adoption

Infectious Diseases

#### 2.3.16. Tenofovir Alafenamide / Emtricitabine - EMEA-001577-PIP02-14-M04

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

Day 60 opinion

**Action:** For adoption

Infectious Diseases

#### 2.3.17. Tenofovir alafenamide / Emtricitabine / Bictegravir - EMEA-001766-PIP01-15-M03

Gilead Sciences International Ltd.; Treatment of Human immunodeficiency virus [HIV]  
disease resulting in other conditions / treatment of adults and paediatrics aged less than 2  
years weighing more than 4 kg infected with human immunodeficiency virus-1 (HIV-1)  
without any known mutations associated with resistance to the individual components

Day 60 opinion

**Action:** For adoption

Infectious Diseases

#### 2.3.18. Cannabidiol - Orphan - EMEA-001964-PIP01-16-M03

GW Pharma (International) B.V.; Lennox Gastaut Syndrome / Tuberous Sclerosis Complex /  
Infantile Spasms / Dravet Syndrome / Treatment of Seizures

Day 60 opinion

**Action:** For adoption

Neurology

### 2.3.19. Lasmiditan - EMEA-002166-PIP01-17-M05

---

Eli Lilly and Company Limited; Migraine with and without aura / Migraine with and without aura

Day 60 opinion

**Action:** For adoption

Neurology

### 2.3.20. Ofatumumab - EMEA-002397-PIP01-18-M01

---

Novartis Europharm Limited; Treatment of multiple sclerosis / Treatment of relapsing remitting multiple sclerosis

Day 60 opinion

**Action:** For adoption

Neurology

### 2.3.21. Pitolisant - Orphan - EMEA-001176-PIP01-11-M06

---

BIOPROJET PHARMA; Narcolesy / Narcolepsy with or without cataplexy

Day 60 opinion

**Action:** For adoption

Neurology

### 2.3.22. Siponimod hemifumarate - EMEA-000716-PIP01-09-M03

---

Novartis Europharm Ltd; Treatment of multiple sclerosis / Treatment of children/adolescent patients (10-18 years old) with relapsing forms of multiple sclerosis

Day 60 opinion

**Action:** For adoption

Neurology

### 2.3.23. Brentuximab vedotin - Orphan - EMEA-000980-PIP01-10-M07

---

Takeda Pharma A/S; Treatment of Hodgkin lymphoma / Treatment of paediatric patients with newly diagnosed relapse or refractory Hodgkin lymphoma (from 5 years of age)

Day 60 opinion

**Action:** For adoption

Oncology

#### 2.3.24. Crizotinib - EMEA-001493-PIP03-18-M01

---

Pfizer Europe MA EEIG; Inflammatory myofibroblastic tumour (IMT) / Anaplastic large cell lymphoma (ALCL) / Treatment of paediatric patients that are able to swallow capsules (age range: 6 years to less than 18 years of age) with relapsed/refractory systemic ALK-positive ALCL / Treatment of paediatric patients that are able to swallow capsules (age range: 6 years to less than 18 years of age) with unresectable or relapsed/refractory ALK-positive IMT / Treatment of paediatric patients with relapsed/refractory systemic ALK-positive ALCL / Treatment of paediatric patients with unresectable or relapsed/refractory ALK-positive IMT

Day 60 opinion

**Action:** For adoption

Oncology

#### 2.3.25. Eribulin - EMEA-001261-PIP01-11-M06

---

Eisai GmbH; Soft Tissue Sarcoma

Day 60 opinion

**Action:** For adoption

Oncology

#### 2.3.26. Fosdenopterin - Orphan - EMEA-001491-PIP01-13-M01

---

Origin Biosciences, Inc.; Treatment of molybdenum cofactor deficiency type A

Day 60 opinion

**Action:** For adoption

Other

#### 2.3.27. Human thrombin (component 2) / Human fibrinogen (component 1) - EMEA-001598-PIP01-13-M03

---

Instituto Grifols, S.A.; Treatment of haemorrhage resulting from a surgical procedure / Supportive treatment in surgery where standard surgical techniques are insufficient for improvement of haemostasis, and as a suture support in vascular surgery

Day 60 opinion

**Action:** For adoption

Other

#### 2.3.28. Human Thrombin / Human Fibrinogen - EMEA-001149-PIP01-11-M06

---

Omrix Biopharmaceuticals N.V.; Treatment of haemorrhage resulting from a surgical procedure / Treatment of cerebrospinal fluid leakage resulting from a surgical procedure / supportive treatment in surgery where standard surgical techniques are insufficient, for

improvement of haemostasis / suture line sealing in dura mater closure / supportive treatment in surgery where standard surgical techniques are insufficient, for improvement of haemostasis

Day 60 opinion

**Action:** For adoption

Other

---

2.3.29. [In vitro expanded autologous human articular chondrocytes - EMEA-001823-PIP01-15-M02](#)

---

TETEC Tissue Engineering Technologies AG; Treatment of cartilage disorders

Day 60 opinion

**Action:** For adoption

Other

---

2.3.30. [In vitro expanded autologous human articular chondrocytes - EMEA-002217-PIP01-17-M01](#)

---

TETEC Tissue Engineering Technologies AG; Treatment of cartilage disorders

Day 60 opinion

**Action:** For adoption

Other

---

2.3.31. [Vortioxetine - EMEA-000455-PIP02-10-M07](#)

---

H. Lundbeck A/S; Major Depressive Disorder

Day 60 opinion

**Action:** For adoption

Psychiatry

---

2.3.32. [Daprodustat - EMEA-001452-PIP01-13-M03](#)

---

GlaxoSmithKline Trading Services Limited; Treatment of anaemia associated with chronic kidney disease

Day 60 opinion

**Action:** For adoption

Uro-nephrology / Haematology-Hemostaseology

---

2.3.33. [Split influenza virus, inactivated containing antigens equivalent to the B-like strain \(Yamagata lineage\) / Split influenza virus, inactivated containing antigens equivalent to the B-like strain \(Victoria lineage\) / Split influenza virus, inactivated](#)

---

containing antigens equivalent to the A/H3N2-like strain / Split influenza virus, inactivated containing antigens equivalent to the A/H1N1-like strain - EMEA-002359-PIP01-18-M02

---

Sanofi Pasteur; Prevention of influenza infection

Day 60 opinion

**Action:** For adoption

Vaccines

## 2.4. Opinions on Re-examinations

### 2.4.1. Adeno-associated viral vector serotype 9 containing the human mini-dystrophin gene (PF-06939926) - Orphan - EMEA-002741-PIP01-20

---

Pfizer Europe MA EEIG; Treatment of Duchenne Muscular Dystrophy

Day 30 opinion

**Action:** For adoption, Oral explanation to be held on 9 December 2020 at 14:00

Neurology

## 2.5. Opinions on Review of Granted Waivers

No items

## 2.6. Finalisation and adoption of opinions

### 2.6.1. BNT162 (mRNA S protein) - EMEA-002861-PIP02-20

---

Biontech; SARS-CoV-2 (COVID-19) infection prevention

Day 30 Opinion

**Action:** For information. Adopted via written procedure on 26 November 2020

Vaccines

### 2.6.2. CX-024414 - mRNA that encodes for the pre-fusion stabilized Spike glycoprotein of 2019-novel Coronavirus - EMEA-002893-PIP01-20

---

Moderna Therapeutics Inc; Prevention of COVID-19 / Active immunisation against SARS-CoV-2

Day 30 Opinion

**Action:** For information. Adopted via written procedure on 30 November 2020

Vaccines

## 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. Daridorexant (hydrochloride) - EMEA-C1-002121-PIP03-19

---

Idorsia Pharmaceuticals Deutschland GmbH; Treatment of insomnia

Day 30 discussion

**Action:** For information

Psychiatry

### 2.7.2. Migalastat (hydrochloride) - EMEA-C1-001194-PIP01-11-M04

---

Amicus Therapeutics Europe Limited; Treatment of Fabry disease

Day 30 discussion

**Action:** For information

Endocrinology-Gynaecology-Fertility-Metabolism

### 2.7.3. Ivacaftor / tezacaftor / elexacaftor - EMEA-C3-002324-PIP01-17-M01

---

Vertex Pharmaceuticals (Europe) Limited; Treatment of cystic fibrosis

Day 30 discussion

**Action:** For information

Other

### 2.7.4. Ruxolitinib (phosphate) - EMEA-C1-000901-PIP03-16-M01

---

Novartis Europharm Limited; Treatment of acute Graft versus Host Disease (aGvHD)

Day 30 discussion

**Action:** For information

Oncology

### 2.7.5. Dulaglutide - EMEA-C2-000783-PIP01-09-M05

---

Eli Lilly & Company Limited; Treatment of type 2 diabetes mellitus

Day 30 discussion

**Action:** For information

Endocrinology-Gynaecology-Fertility-Metabolism

### 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. EMEA-002329-PIP02-20

---

Treatment of dermatitis and eczema / Treatment of chronic hand eczema / Treatment of atopic dermatitis

Day 90 discussion

**Action:** For discussion

Dermatology

##### 3.1.2. Crinecerfont; 2-Thiazolamine, 4-(2-chloro-4-methoxy-5-methylphenyl)-N-[(1S)-2-cyclopropyl-1-(3-fluoro-4-methylphenyl)ethyl]-5-methyl-N-2-propyn-1-yl; - Orphan - EMEA-002700-PIP01-19

---

Neurocrine Therapeutics Ltd; Treatment of congenital adrenal hyperplasia

Day 90 discussion

**Action:** For discussion

Endocrinology-Gynaecology-Fertility-Metabolism

##### 3.1.3. EMEA-002773-PIP01-20

---

Treatment of non-alcoholic steatohepatitis (NASH)

Day 90 discussion

**Action:** For discussion

Gastroenterology-Hepatology

##### 3.1.4. Crovalimab - EMEA-002709-PIP01-19

---

Treatment of paroxysmal nocturnal hemoglobinuria (PNH)

Day 90 discussion

**Action:** For discussion

Haematology-Hemostaseology

##### 3.1.5. Rozibafusp alfa - EMEA-002815-PIP01-20

---

Systemic lupus erythematosus

---



Day 90 discussion

**Action:** For discussion

Immunology-Rheumatology-Transplantation

### 3.1.6. Secukinumab - EMEA-000380-PIP06-19

---

Treatment of lupus nephritis

Day 90 discussion

**Action:** For discussion

Immunology-Rheumatology-Transplantation

### 3.1.7. Tacrolimus - EMEA-001642-PIP02-20

---

Solid organ transplant rejection / Prophylaxis of transplant rejection in liver, kidney or heart allograft recipients (children aged from birth to less than 18 years) / Treatment of allograft rejection resistant to treatment with other immunosuppressive medical products in children aged from birth to less than 18 years

Day 90 discussion

**Action:** For discussion

Immunology-Rheumatology-Transplantation

### 3.1.8. Telitacicept - EMEA-002824-PIP01-20

---

Treatment of systemic lupus erythematosus

Day 90 discussion

**Action:** For discussion

Immunology-Rheumatology-Transplantation

### 3.1.9. Bimekizumab - EMEA-002189-PIP04-20

---

Treatment of hidradenitis suppurativa / Treatment of moderate to severe hidradenitis suppurativa in adolescents from 12 years of age

Day 90 discussion

**Action:** For discussion

Immunology-Rheumatology-Transplantation / Dermatology

### 3.1.10. Rimegepant - EMEA-002812-PIP02-20

---

Acute treatment of migraine

Day 90 discussion

**Action:** For discussion

Neurology

### 3.1.11. Imetelstat - Orphan - EMEA-001910-PIP03-20

---

Geron Corporation; Treatment of Acute Myeloid Leukemia (AML) / Treatment of Myelodysplastic Syndromes (MDS), including Juvenile Myelomonocytic Leukemia (JMML) / Treatment of pediatric patients with relapsed or refractory AML or MDS, including JMML, from 28 days to less than 18 years of age

Day 90 discussion

**Action:** For discussion

Oncology

### 3.1.12. Linear single strand of deoxyribonucleic acid (encoding human retinitis pigmentosa GTPase regulator [RPGR]) packaged in a recombinant adeno-associated virus protein capsid of serotype 5 (AAV5-hRKp.RPGR) - Orphan - EMEA-002827-PIP01-20

---

MeiraGTx UK II Ltd; Retinitis pigmentosa / RPGR mutation-associated X-linked retinitis pigmentosa

Day 90 discussion

**Action:** For discussion

Ophthalmology

### 3.1.13. Seltorexant - EMEA-002746-PIP01-20

---

Major Depressive Disorder (MDD)

Day 90 discussion

**Action:** For discussion

Psychiatry

### 3.1.14. Respiratory Syncytial Virus (RSV) PreF3 recombinant Fusion protein - EMEA-002821-PIP01-20

---

Prevention of RSV- associated lower respiratory tract illness through maternal immunization / Active immunization of pregnant women during the second and third trimester of pregnancy to prevent medically assessed respiratory syncytial virus (RSV) -associated lower respiratory tract illness (LRTI) in infants by transfer of maternal antibodies

Day 90 discussion

**Action:** For discussion

Vaccines

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

---

Atopic dermatitis / Treatment of moderate to severe atopic dermatitis

Day 60 discussion

**Action:** For discussion

Dermatology

### 3.1.16. Semaglutide - EMEA-001441-PIP05-20

---

Treatment of non-alcoholic fatty liver disease (NAFLD)

Day 60 discussion

**Action:** For discussion

Gastroenterology-Hepatology

### 3.1.17. Fenebrutinib - EMEA-002349-PIP03-20

---

Treatment of multiple sclerosis / Treatment of relapsing multiple sclerosis in patients 10 years of age to less than 18 years of age

Day 60 discussion

**Action:** For discussion

Immunology-Rheumatology-Transplantation

### 3.1.18. Trimodulin - EMEA-002883-PIP01-20

---

Treatment of bacterial infections / Treatment of community-acquired sepsis / Treatment of neonatal sepsis / Treatment of severe community-acquired pneumonia/ Treatment of community-acquired septic shock / Treatment of community-acquired severe sepsis

Day 60 discussion

**Action:** For discussion

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

### 3.1.19. Zidebactam / cefepime - EMEA-002892-PIP01-20

---

Treatment of complicated urinary tract infections (cUTI)

Day 60 discussion

**Action:** For discussion

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

### 3.1.20. Ravulizumab - EMEA-001943-PIP03-20

---

Acetylcholine receptor-antibody positive generalized myasthenia gravis / Treatment of acetylcholine receptor-antibody positive generalized myasthenia gravis

Day 60 discussion

**Action:** For discussion

Neurology

### 3.1.21. Allogeneic anti-CD19 CAR T cells produced using CRISPR/Cas9 to disrupt the T cell receptor alpha constant (TRAC) and $\beta$ 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

---

B cell lymphoblastic leukemia/lymphoma / Mature B cell neoplasms / Treatment of relapsed/refractory B cell ALL / Treatment of relapsed/refractory B cell NHL

Day 60 discussion

**Action:** For discussion

Oncology

### 3.1.22. Autologous peripheral blood T cells CD4- and CD8-selected and CD3- and CD28-activated transduced with retroviral vector expressing anti-CD19 CD28/CD3-zeta chimeric antigen receptor and cultured - Orphan - EMEA-001862-PIP03-20

---

Kite Pharma EU B.V.; Treatment of mature B-cell neoplasms / Treatment of pediatric and adolescent subjects with relapsed or refractory B-cell non-Hodgkin lymphoma (NHL)

Day 60 discussion

**Action:** For discussion

Oncology

### 3.1.23. Ribitol - EMEA-002887-PIP01-20

---

Treatment of Limb-Girdle Muscular Dystrophy / Treatment of paediatric patients aged 5 years and older with a genetically confirmed diagnosis of Limb-Girdle Muscular Dystrophy type 2i (LGMD2I)/LGMD R9-fukutin related

Day 60 discussion

**Action:** For discussion

Other

### 3.1.24. COVID-19 Vaccine - EMEA-002862-PIP01-20

---

COVID-19 / For the prevention of COVID-19

Day 60 discussion

**Action:** For discussion

Vaccines

### 3.1.25. Hydrochlorothiazide / Amlodipine / Ramipril - EMEA-002906-PIP01-20

Treatment of hypertension

Day 30 discussion

**Action:** For discussion

Cardiovascular Diseases

### 3.1.26. Olpasiran - EMEA-002910-PIP01-20

Prevention of cardiovascular events

Day 30 discussion

**Action:** For discussion

Cardiovascular Diseases

### 3.1.27. Canakinumab - EMEA-000060-PIP09-20

Schnitzler Syndrome

Day 30 discussion

**Action:** For discussion

Immunology-Rheumatology-Transplantation

### 3.1.28. Secukinumab - EMEA-000380-PIP07-20

Thyroid eye disease

Day 30 discussion

**Action:** For discussion

Immunology-Rheumatology-Transplantation

### 3.1.29. Anti-C1s Humanized IgG4 Monoclonal Antibody - EMEA-002903-PIP01-20

Immune Thrombocytopenia Purpura

Day 30 discussion

**Action:** For discussion

Immunology-Rheumatology-Transplantation / Haematology-Hemostaseology / Neurology

3.1.30. [Allogeneic multi-virus specific T lymphocytes targeting BK Virus, cytomegalovirus, human herpes virus-6, Epstein Barr virus and adenovirus - Orphan - EMEA-002908-PIP01-20](#)

---

AlloVir International DAC; Treatment of viral diseases in haematopoietic stem cell transplantation / Treatment of virus-associated haemorrhagic cystitis in allogeneic haematopoietic stem cell transplantation recipients / Pre-emptive treatment of cytomegalovirus disease in allogeneic haematopoietic stem cell transplantation recipients / Pre-emptive treatment of adenovirus disease in allogeneic haematopoietic stem cell transplantation recipients

Day 30 discussion

**Action:** For discussion

Infectious Diseases

3.1.31. [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) / For the treatment of severe or critical COVID-19 in hospitalised children from 32 weeks gestational age (GA) to less than 18 years of age

Day 30 discussion

**Action:** For discussion

Infectious Diseases

3.1.32. [Potassium Bitartrate / Citric Acid / L-Lactic Acid - EMEA-002917-PIP01-20](#)

---

Prevention of urogenital *Chlamydia trachomatis* (CT) infection and *Neisseria gonorrhoeae* (GC) infection in females

Day 30 discussion

**Action:** For discussion

Infectious Diseases

3.1.33. [Edaravone - Orphan - EMEA-002897-PIP01-20](#)

---

Mitsubishi Tanabe Pharma GmbH; Amyotrophic Lateral Sclerosis

Day 30 discussion

**Action:** For discussion

Neurology

3.1.34. [Eptinezumab - EMEA-002243-PIP02-20](#)

---

Episodic cluster headache / Prophylaxis

---

Day 30 discussion

**Action:** For discussion

Neurology

---

### 3.1.35. [Omaveloxolone - Orphan - EMEA-002487-PIP01-18](#)

---

Reata Pharmaceuticals Inc.; Treatment of Friedreich's ataxia

Day 30 discussion

**Action:** For discussion

Neurology

---

### 3.1.36. [Ublituximab - EMEA-002889-PIP02-20](#)

---

Relapsing forms of multiple sclerosis (RMS) / Treatment

Day 30 discussion

**Action:** For discussion

Neurology

---

### 3.1.37. [Vatiquinone - Orphan - EMEA-001238-PIP02-20](#)

---

PTC Therapeutics International Limited; Treatment of mitochondrial epilepsy

Day 30 discussion

**Action:** For discussion

Neurology

---

### 3.1.38. [Alpelisib - EMEA-002016-PIP04-20](#)

---

ovarian cancer

Day 30 discussion

**Action:** For discussion

Oncology

---

### 3.1.39. [Autologous CD4+ and CD8+ T cells genetically modified with a lentiviral vector encoding a B cell maturation antigen-specific chimeric antigen receptor - EMEA-002909-PIP01-20](#)

---

Treatment of mature B cell neoplasms / Not applicable

Day 30 discussion

**Action:** For discussion

Oncology

#### 3.1.40. Epcoritamab - EMEA-002907-PIP01-20

---

Treatment of mature B-cell lymphoma / Treatment of paediatric patients with relapsed/refractory aggressive mature B-cell lymphoma

Day 30 discussion

**Action:** For discussion

Oncology

#### 3.1.41. EMEA-002895-PIP01-20

---

Treatment of macular oedema due to central or tributary (branch) retinal vein occlusion / Treatment of retinopathy of prematurity / Treatment of diabetic retinopathy / Treatment of choroidal neovascularisation

Day 30 discussion

**Action:** For discussion

Ophthalmology

#### 3.1.42. Isopropyl alcohol / Povidone-iodine - EMEA-002902-PIP01-20

---

Prevention of infection prior to invasive procedures

Day 30 discussion

**Action:** For discussion

Other

#### 3.1.43. Iptacopan - EMEA-002705-PIP04-20

---

Atypical haemolytic uremic syndrome

Day 30 discussion

**Action:** For discussion

Other / Haematology-Hemostaseology

#### 3.1.44. Dupilumab - EMEA-001501-PIP08-20

---

Chronic Rhinosinusitis without Nasal Polyposis / Chronic Rhinosinusitis without Nasal Polyposis

Day 30 discussion

**Action:** For discussion

Oto-rhino-laryngology



### 3.1.45. Brensocatib - EMEA-002905-PIP01-20

---

Non-cystic fibrosis bronchiectasis (NCFBE) / Treatment of NCFBE for reducing exacerbations.

Day 30 discussion

**Action:** For discussion

Pneumology - Allergology

### 3.1.46. Recombinant human pentraxin-2 - Orphan - EMEA-002878-PIP02-20

---

Roche Registration GmbH; Idiopathic pulmonary fibrosis

Day 30 discussion

**Action:** For discussion

Pneumology - Allergology

### 3.1.47. Single chain urokinase plasminogen activator (scuPA) - Orphan - EMEA-002896-PIP01-20

---

Lung Therapeutics, Inc.; Treatment of infectious pleural effusion / Treatment of infected, non-draining pleural effusions including complicated parapneumonic pleural effusion (CPE), empyema and other forms of pleural space infection

Day 30 discussion

**Action:** For discussion

Pneumology - Allergology

### 3.1.48. Thienopyrimidine Derivative - EMEA-002901-PIP01-20

---

Treatment of chronic fibrosing interstitial lung diseases (ILD)

Day 30 discussion

**Action:** For discussion

Pneumology - Allergology

### 3.1.49. Bardoxolone methyl - Orphan - EMEA-002488-PIP01-18

---

Reata Pharmaceuticals Inc.; Treatment of Alport syndrome / Treatment of Alport syndrome

Day 30 discussion

**Action:** For discussion

Uro-nephrology

### 3.1.50. Respiratory Syncytial Virus (RSV) PreF3 recombinant Fusion protein - EMEA-002904-PIP01-20

---

Prevention of lower respiratory tract disease caused by respiratory syncytial virus / Active immunization in the prevention of respiratory syncytial virus (RSV)-associated lower respiratory tract disease (LRTD) in subjects aged 2 to 18 years who are at high risk of RSV-associated LRTD

Day 30 discussion

**Action:** For discussion

Vaccines

### 3.1.51. - EMEA-002880-PIP01-20

---

Active immunisation for the prevention of COVID-19

Day 30 discussion

**Action:** For discussion

Vaccines / Infectious Diseases

### 3.1.52. Edasalonexent [N-(2-((4Z,7Z,10Z,13Z,16Z,19Z)-docosa-4,7,10,13,16,19-hexaenamido)ethyl)-2-hydroxybenzamide] - Orphan - EMEA-001960-PIP03-20

---

Catabasis Pharmaceuticals Inc.; Duchenne Muscular Dystrophy / Treatment of Duchenne Muscular Dystrophy

Day 60 discussion

**Action:** For discussion

Neurology

Note: Procedure withdrawn on 24 November 2020

### 3.1.53. Baricitinib - EMEA-001220-PIP07-20

---

Treatment of COVID-19

Day 30 discussion

**Action:** For discussion

Vaccines / Infectious Diseases

### 3.1.54. Otilimab - EMEA-001882-PIP03-20

---

Treatment of COVID-19

Day 30 discussion

**Action:** For discussion

Vaccines / Infectious Diseases

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## 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. Dapagliflozin - EMEA-C-000694-PIP01-09-M08

---

Astrazeneca AB; Treatment of type 2 diabetes

Day 30 discussion

**Action:** For discussion

Endocrinology-Gynaecology-Fertility-Metabolism

### 3.2.2. Oseltamivir phosphate - EMEA-C-000365-PIP01-08-M11

---

Roche Registration GmbH; Treatment and prevention of influenza

Day 30 discussion

**Action:** For discussion

Infectious Diseases

### 3.2.3. Copanlisib - EMEA-C1-001757-PIP02-15-M02

---

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

Day 30 discussion

**Action:** For discussion

Oncology

### 3.2.4. Daunorubicin (liposomal combination)/cytarabine (liposomal combination) - EMEA-C2-001858-PIP02-16-M03

---

Jazz Pharmaceuticals Ireland Limited; Treatment of acute myeloid leukaemia

Day 30 discussion

**Action:** For discussion

Oncology

### 3.3. Discussions on Modification of an Agreed Paediatric Investigation Plan

#### 3.3.1. Lamivudine (3TC) / Abacavir (ABC) / Dolutegravir (DTG) - EMEA-001219-PIP01-11-M05

---

ViiV Healthcare UK Limited; Treatment of Human Immunodeficiency Virus (HIV-1) infection / Treatment Human Immunodeficiency Virus (HIV-1) infection in paediatric population

Day 30 discussion

**Action:** For discussion

#### 3.3.2. Enalapril maleate - EMEA-001706-PIP01-14-M03

---

Proveca Pharma Limited; Treatment of Heart Failure

Day 30 discussion

**Action:** For discussion

Cardiovascular Diseases

#### 3.3.3. 6-cyclopropaneamido-4-{{2-methoxy-3-(1-methyl-1H-1,2,4 triazol-3-yl)phenyl}amino}-N-(2H3)methylpyridazine-3-carboxamide - EMEA-002350-PIP01-18-M01

---

Bristol-Myers Squibb International Corporation; Treatment of psoriasis / Treatment of moderate to severe plaque psoriasis in paediatric patients 6 years of age and older

Day 30 discussion

**Action:** For discussion

Dermatology

#### 3.3.4. Tildrakizumab - EMEA-001451-PIP01-13-M01

---

Almirall, S.A; Psoriasis / Treatment of moderate to severe plaque psoriasis in patients from 6 to less than 18 years of age who are candidates for systemic therapy

Day 30 discussion

**Action:** For discussion

Dermatology

#### 3.3.5. Empagliflozin - EMEA-000828-PIP01-09-M08

---

Boehringer Ingelheim International GmbH; Treatment of type 2 diabetes mellitus

Day 30 discussion

**Action:** For discussion

Endocrinology-Gynaecology-Fertility-Metabolism

---

### 3.3.6. Evinacumab - EMEA-002298-PIP01-17-M02

---

Regeneron Ireland DAC; Treatment of elevated cholesterol / Treatment of homozygous familial hypercholesterolemia (HoFH)

Day 30 discussion

**Action:** For discussion

Endocrinology-Gynaecology-Fertility-Metabolism

### 3.3.7. Exenatide - EMEA-000689-PIP01-09-M11

---

AstraZeneca AB; Non Insulin dependent diabetes mellitus (excluding treatment with thiazolidinediones), Non Insulin dependent diabetes mellitus (treatment including thiazolidinediones), Non Insulin dependent diabetes mellitus - in combination with insulin (with or without oral antidiabetics) / Treatment of Type 2 Diabetes Mellitus

Day 30 discussion

**Action:** For discussion

Endocrinology-Gynaecology-Fertility-Metabolism

### 3.3.8. Linagliptin - EMEA-000498-PIP01-08-M09

---

Boehringer Ingelheim International GmbH; Treatment of type 2 diabetes mellitus

Day 30 discussion

**Action:** For discussion

Endocrinology-Gynaecology-Fertility-Metabolism

### 3.3.9. Ozanimod hydrochloride - EMEA-001710-PIP04-17-M02

---

Celgene Europe B.V.; Treatment of Crohn's disease / Treatment of moderate to severe active Crohn's disease

Day 30 discussion

**Action:** For discussion

Gastroenterology-Hepatology

### 3.3.10. Ustekinumab - EMEA-000311-PIP04-13-M04

---

Janssen-Cilag International NV; Crohn's Disease / Treatment of Crohn's Disease

Day 30 discussion

**Action:** For discussion

Gastroenterology-Hepatology

### 3.3.11. Ustekinumab - EMEA-000311-PIP05-17-M02

---

Janssen-Cilag International NV; Ulcerative Colitis / Treatment of Ulcerative Colitis

Day 30 discussion

**Action:** For discussion

Gastroenterology-Hepatology

### 3.3.12. Roxadustat - EMEA-001557-PIP01-13-M05

---

Astellas Pharma Europe B.V.; Treatment of anaemia due to chronic disorders

Day 30 discussion

**Action:** For discussion

Haematology-Hemostaseology

### 3.3.13. Sarilumab - EMEA-001045-PIP01-10-M02

---

sanofi-aventis recherche & développement; Treatment of chronic idiopathic arthritis (including rheumatoid arthritis, spondylarthritis, psoriatic arthritis and juvenile idiopathic arthritis)

Day 30 discussion

**Action:** For discussion

Immunology-Rheumatology-Transplantation

### 3.3.14. Cabotegravir - EMEA-001418-PIP02-15-M01

---

ViiV Healthcare UK Limited; Prevention of human immunodeficiency virus (HIV-1) infection / In combination with safer sex practices for pre-exposure prophylaxis to reduce the risk of HIV-1 acquisition in sexually active adolescents at high risk, from 12 to less than 18 years of age

Day 30 discussion

**Action:** For discussion

Infectious Diseases

### 3.3.15. Islatravir / Doravirine - EMEA-002707-PIP01-19-M01

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Merck Sharp & Dohme (Europe), Inc.; Treatment of human immunodeficiency virus-1 (HIV-1) infection

Day 30 discussion

**Action:** For discussion

Infectious Diseases

### 3.3.16. Remdesivir - EMEA-002826-PIP01-20-M01

---

Gilead Sciences International Ltd.; Coronavirus disease 2019 (COVID-19) / treatment of paediatric patients aged from birth to < 18 years (weighing < 40kg) with coronavirus disease 2019 (COVID-19)

Day 30 discussion

**Action:** For discussion

Infectious Diseases

### 3.3.17. Efgartigimod alfa - Orphan - EMEA-002597-PIP01-19-M01

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argenx BV; Generalised myasthenia gravis

Day 30 discussion

**Action:** For discussion

Neurology

### 3.3.18. Eladocagene exuparvovec - Orphan - EMEA-002435-PIP01-18-M02

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PTC Therapeutic International Limited; Aromatic L-Amino Acid Decarboxylase (AADC) Deficiency / Treatment of Aromatic L-Amino Acid Decarboxylase (AADC) Deficiency

Day 30 discussion

**Action:** For discussion

Neurology

### 3.3.19. Bintrafusp alfa - Orphan - EMEA-002586-PIP01-19-M01

---

Merck Europe B.V.; Treatment of all conditions included in the category of malignant neoplasms (except central nervous system neoplasms, haematopoietic and lymphoid tissue neoplasms) / Treatment of paediatric patients from 0 to <18 years of age with solid malignant tumors

Day 30 discussion

**Action:** For discussion

Oncology

### 3.3.20. Cobimetinib - EMEA-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 / Treatment of children with a paediatric solid malignant tumour with known or expected Ras, Raf or MEK pathway activation, at first relapse or refractory to initial treatment.

Day 30 discussion

**Action:** For discussion

Oncology

### 3.3.21. Durvalumab - EMEA-002028-PIP01-16-M02

---

AstraZeneca AB; Treatment of malignant neoplasms of haematopoietic and lymphoid tissue., Treatment of all conditions included in the category of malignant neoplasms (except central nervous system, haematopoietic and lymphoid tissue) / Treatment of paediatric patients from birth to less than 18 years old with a paediatric haematological malignancy., Treatment of paediatric patients from birth to less than 18 years old with a paediatric solid tumour

Day 30 discussion

**Action:** For discussion

Oncology

### 3.3.22. Ibrutinib - Orphan - EMEA-001397-PIP03-14-M05

---

Janssen-Cilag International N.V.; Treatment of mature B-cell neoplasm / Treatment of children from 1 year to less than 18 years of age with newly-diagnosed and relapsed/refractory mature B-cell lymphoma, that is, diffuse large B-cell lymphoma or Burkitt and Burkitt-like lymphoma

Day 30 discussion

**Action:** For discussion

Oncology

### 3.3.23. Idecabtagene vicleucel - Orphan - EMEA-002369-PIP01-18-M02

---

Celgene Europe B.V.; Treatment of mature B-cell neoplasms / Treatment of paediatric patients with relapsed or refractory BCMA+ B-cell non-Hodgkin lymphoma

Day 30 discussion

**Action:** For discussion

Oncology

### 3.3.24. Larotrectinib - EMEA-001971-PIP02-16-M03

---

Bayer AG; Treatment of all conditions included in the category of malignant neoplasms (except central nervous system tumours, haematopoietic and lymphoid tissue neoplasms) / Treatment of paediatric patients from birth to less than 18 years of age with advanced solid tumours harbouring an NTRK fusion

Day 30 discussion

**Action:** For discussion

Oncology



### 3.3.25. Larotrectinib - EMEA-001971-PIP03-18-M01

---

Bayer AG; Treatment of malignant neoplasms of the central nervous system / Treatment of paediatric patients from birth to less than 18 years of age with a primary CNS tumour harbouring an NTRK fusion

Day 30 discussion

**Action:** For discussion

Oncology

### 3.3.26. Midostaurin - Orphan - EMEA-000780-PIP01-09-M06

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Novartis Europharm Limited; Acute myeloid leukaemia / Mast cell leukaemia / Malignant mastocytosis / Treatment of paediatric patients with FLT3 mutated AML, newly diagnosed

Day 30 discussion

**Action:** For discussion

Oncology

### 3.3.27. Nivolumab / relatlimab - EMEA-002727-PIP01-19-M01

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Bristol-Myers Squibb International Corporation; Treatment of melanoma / Relatlimab/nivolumab fixed dose combination for treatment of unresectable or metastatic melanoma in patients from 12 to 18 years

Day 30 discussion

**Action:** For discussion

Oncology

### 3.3.28. Palbociclib - EMEA-002146-PIP01-17-M02

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Pfizer Europe MA EEIG; Treatment of Ewing sarcoma / treatment of refractory or recurrent Ewing sarcoma

Day 30 discussion

**Action:** For discussion

Oncology

### 3.3.29. Tremelimumab - EMEA-002029-PIP01-16-M02

---

AstraZeneca AB; Treatment of malignant neoplasms of haematopoietic and lymphoid tissue / Treatment of all conditions included in the category of malignant neoplasms (except central nervous system, haematopoietic and lymphoid tissue) / Treatment of paediatric patients from birth to less than 18 years old with a paediatric haematological malignancy / Treatment of paediatric patients from birth to less than 18 years old with a paediatric solid tumour

Day 30 discussion

**Action:** For discussion

Oncology

### 3.3.30. Gilteritinib (as fumarate) - Orphan - EMEA-002064-PIP01-16-M03

---

Astellas Pharma Europe B.V.; Treatment of acute myeloid leukemia / Treatment of patients from 6 months to less than 18 years of age with relapsed or refractory FLT3/ITD positive acute myeloid leukaemia or newly-diagnosed FLT3/ITD positive acute myeloid leukaemia

Day 30 discussion

**Action:** For discussion

Oncology / Haematology-Hemostaseology

### 3.3.31. Vamorolone - Orphan - EMEA-001794-PIP02-16-M03

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ReveraGen BioPharma Ltd; Treatment of Duchenne muscular dystrophy

Day 30 discussion

**Action:** For discussion

Other

### 3.3.32. Ponesimod - EMEA-000798-PIP01-09-M03

---

Janssen-Cilag International NV; Multiple Sclerosis

Day 30 discussion

**Action:** For discussion

Other / Neurology

### 3.3.33. Bupivacaine - EMEA-000877-PIP03-17-M02

---

Pacira Ltd; Postsurgical analgesia

Day 30 discussion

**Action:** For discussion

Pain

### 3.3.34. Glycopyrronium bromide / Formoterol fumarate dihydrate / Beclometasone dipropionate - EMEA-001875-PIP02-18-M03

---

Chiesi Farmaceutici S.p.A.; Treatment of asthma / Regular treatment of asthma in patients not controlled with medium-high doses of inhaled corticosteroids and long-acting beta2-agonists

Day 30 discussion

**Action:** For discussion

Pneumology - Allergology

### 3.3.35. Interferon beta-1a - Orphan - EMEA-002238-PIP01-17-M01

Faron Pharmaceuticals Ltd.; Treatment of Acute Respiratory Distress Syndrome (ARDS)

Day 30 discussion

**Action:** For discussion

Pneumology - Allergology

### 3.3.36. Vilanterol / fluticasone furoate - EMEA-000431-PIP01-08-M12

Glaxo Group Limited; Mixed Asthma / Treatment of asthma where use of a combination product (long acting beta agonist and inhaled corticosteroid) is appropriate

Day 30 discussion

**Action:** For discussion

Pneumology - Allergology

### 3.3.37. Agomelatine - EMEA-001181-PIP01-11-M06

Les Laboratoires Servier; Major Depressive Episodes

Day 30 discussion

**Action:** For discussion

Psychiatry

### 3.3.38. Cariprazine (hydrochloride) - EMEA-001652-PIP01-14-M03

Gedeon Richter Plc.; Schizophrenia

Day 30 discussion

**Action:** For discussion

Psychiatry

### 3.3.39. 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) - Orphan - EMEA-002493-PIP01-18-M02

Dicerna Ireland Limited; Primary hyperoxaluria

Day 30 discussion

**Action:** For discussion

Uro-nephrology

## 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 letters of intent received for submission of applications with start of procedure 4 January 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

No items

### 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

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

No items

## 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)

---

**Action:** For information

### 9.3. Coordination with EMA Working Parties/Working Groups/Drafting Groups

#### 9.3.1. Non-clinical Working Group: D30 Products identified

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PDCO member: Karen van Malderen

**Action:** For information

#### 9.3.2. Formulation Working Group

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PDCO member: Brian Aylward

PDCO Formulation Working Group list of experts for 2021

**Action:** For adoption

### 9.4. Cooperation within the EU regulatory network

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

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**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**

### **9.7.1. PDCO Work Plan 2021**

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PDCO Chair: Koenraad Norga;

**Action:** For discussion

## **9.8. Planning and reporting**

No items

## **10. Any other business**

### **10.1. COVID -19 update**

**Action:** For information

### **10.2. Progress report on EMA/EC paediatric action plan**

**Action:** For information

### **10.3. EMA Business Pipeline activity and Horizon scanning**

**Action:** For information

### **10.4. Big Data Training Signpost**

**Action:** For information

## **11. Breakout sessions**

### **11.1. 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/](http://www.ema.europa.eu/)