



EUROPEAN MEDICINES AGENCY  
SCIENCE MEDICINES HEALTH

20 December 2017  
EMA/PDCO/730337/2017 Corr.<sup>1</sup>  
Inspections, Human Medicines Pharmacovigilance and Committees Division

## Paediatric Committee (PDCO)

Draft agenda for the meeting on 7-10 November 2017

Chair: Dirk Mentzer – Vice-Chair: Koenraad Norga

7 November 2017, 14:00- 19:00, room 3A

8 November 2017, 08:30- 19:00, room 3A

9 November 2017, 08:30- 19:00, room 3A

10 November 2017, 08:30- 13:00, room 3A

### 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 on-going procedures for which a final decision has not yet been adopted. They will become public when adopted or considered public according to the principles stated in the Agency policy on access to documents (EMA/127362/2006).

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<sup>1</sup> Editorial correction under 3.1.16.



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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 7-10 November 2017. See November 2017 PDCO minutes (to be published post December 2017 PDCO meeting).

### 1.2. Adoption of agenda

PDCO agenda for 7-10 November 2017.

### 1.3. Adoption of the minutes

PDCO minutes for 10-13 October 2017.

## 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. Brazikumab - EMEA-001929-PIP01-16

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Crohn's disease, Ulcerative colitis

Day 120 opinion

**Action:** For adoption

Gastroenterology-Hepatology

#### 2.1.2. Susoctocog alfa - EMEA-000753-PIP02-16

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Congenital haemophilia A with antibodies (inhibitors) to human factor VIII / Peri-operative management in patients with congenital haemophilia A with antibodies (inhibitors) to human FVIII

Day 120 opinion

**Action:** For adoption

Haematology-Hemostaseology

#### 2.1.3. Risankizumab - EMEA-001776-PIP02-17

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Chronic Idiopathic Arthritis

Day 120 opinion

**Action:** For adoption

Immunology-Rheumatology-Transplantation

#### 2.1.4. Trazodone hydrochloride - EMEA-002142-PIP01-17

Treatment of insomnia

Day 120 opinion

**Action:** For adoption

Neurology

#### 2.1.5. Carotuximab - Orphan - EMEA-002138-PIP01-17

TRACON Pharma Limited; Treatment of soft tissue sarcoma

Day 120 opinion

**Action:** For adoption

Oncology

#### 2.1.6. fully human monoclonal antibody (mAb) directed against the human PD-1 receptor - EMEA-002007-PIPO2-17

Treatment of all conditions included in the category of malignant neoplasms (except haematopoietic and lymphoid tissue) / Treatment of newly diagnosed diffuse intrinsic pontine gliomas (DIPG), newly diagnosed and recurrent high-grade gliomas (HGG)

Day 120 opinion

**Action:** For adoption

Oncology

#### 2.1.7. Vosoritide - Orphan - EMEA-002033-PIP01-16

BioMarin International Limited; Treatment of achondroplasia

Day 120 opinion

**Action:** For adoption

Other

#### 2.1.8. Bupivacaine - EMEA-000877-PIPO2-16

postsurgical analgesia

Day 120 opinion

**Action:** For adoption, Oral Explanation Meeting to be held on 9 November 2017, 15:00-16:00



Pain

### 2.1.9. [Formoterol fumarate / glycopyrronium bromide / budesonide - EMEA-002063-PIP01-16](#)

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Asthma / For the regular treatment of asthma in children 6 to 11 years of age where use of a triple combination medicinal product (ICS, LAMA and LABA) is appropriate: patients not adequately controlled with ICS and another controller such as a LABA or LAMA

Day 120 opinion

**Action:** For adoption

Pneumology - Allergology

### 2.1.10. [Synthetic double-stranded small interfering RNA \(siRNA\) oligonucleotide specific to the mRNA of the caspase 2 gene - EMEA-002224-PIP01-17](#)

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Treatment of optic nerve bleeding and vascular disorders / Treatment of ischaemic optic neuropathy

Day 60 opinion

**Action:** For adoption

Ophthalmology

### 2.1.11. [Sirolimus - Orphan - EMEA-002213-PIP01-17](#)

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Vascular Therapies, Inc.; Prevention of arteriovenous access dysfunction

Day 60 opinion

**Action:** For adoption

Other

## 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. [Avacopan - EMEA-C1-002023-PIP01-16-M01](#)

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ChemoCentryx, Ltd.; Treatment of antineutrophil cytoplasmic antibody (ANCA)-associated vasculitis

Day 60 letter

**Action:** For adoption

Immunology-Rheumatology-Transplantation

### 2.2.2. [Mepolizumab - EMEA-C-000069-PIP02-10-M08](#)

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GSK TRADING SERVICES LIMITED; Treatment of asthma

Day 60 opinion

**Action:** For adoption

Pneumology – Allergology

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### 2.2.3. [L-asparaginase encapsulated in erythrocytes - EMEA-C2-000341-PIP02-09-M04](#)

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ERYTECH Pharma S.A.; Treatment of Acute Lymphoblastic Leukaemia

Day 1 letter

**Action:** For adoption

Oncology

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### 2.2.4. [Fingolimod \(hydrochloride\) - EMEA-C-000087-PIP01-07-M05](#)

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

Day 1 opinion

**Action:** For adoption

Neurology

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

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### 2.3.1. [Recombinant human glutamic acid decarboxylase \(rhGAD65\) - EMEA-000609-PIP01-09-M01](#)

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Diamyd Medical AB; E10 Insulin-dependent diabetes mellitus / Treatment of type 1 Diabetes Mellitus of recent onset

Day 60 opinion

**Action:** For adoption

Endocrinology-Gynaecology-Fertility-Metabolism

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### 2.3.2. [Eluxadoline - EMEA-001579-PIP01-13-M02](#)

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Allergan Limited; Irritable bowel syndrome with diarrhoea

Day 60 opinion

**Action:** For adoption

Gastroenterology-Hepatology

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### 2.3.3. [Human coagulation factor X - Orphan - EMEA-000971-PIP01-10-M03](#)

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Bio Products Laboratory Limited; Hereditary factor X deficiency

Day 60 opinion

**Action:** For adoption

Haematology-Hemostaseology

#### 2.3.4. [Avacopan - Orphan - EMEA-002023-PIP01-16-M02](#)

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ChemoCentryx, Ltd.; Treatment of ANCA-associated vasculitis

Day 60 opinion

**Action:** For adoption

Immunology-Rheumatology-Transplantation

#### 2.3.5. [Human normal immunoglobulin for subcutaneous administration - EMEA-001853-PIP01-15-M01](#)

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Grifols Therapeutics Inc; Treatment for primary immunodeficiency

Day 60 opinion

**Action:** For adoption

Immunology-Rheumatology-Transplantation

#### 2.3.6. [Bezlotoxumab - EMEA-001645-PIP01-14-M02](#)

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Merck Sharp & Dohme (Europe), Inc.; Treatment of Clostridium difficile infection / indicated for the prevention of recurrence of Clostridium difficile infection (CDI) in paediatric patients at high risk for recurrence of CDI

Day 60 opinion

**Action:** For adoption

Infectious Diseases

#### 2.3.7. [Ataluren - Orphan - EMEA-000115-PIP01-07-M09](#)

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PTC Therapeutics International, Limited; Treatment of dystrophinopathy ICD-10: G71.0 Muscular dystrophy [of Duchenne and Becker] / Treatment of nonsense-mutation dystrophinopathy

Day 60 opinion

**Action:** For adoption

Neurology

#### 2.3.8. [Delta-9-tetrahydrocannabinol / Cannabidiol - EMEA-000181-PIP01-08-M04](#)

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GW Pharma Ltd; Spasticity / Intractable spasticity due to cerebral palsy or traumatic CNS injury

Day 60 opinion

**Action:** For adoption

Neurology

### 2.3.9. Blinatumomab - Orphan - EMEA-000574-PIP02-12-M02

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Amgen Europe B.V.; Treatment of Acute Lymphoblastic Leukaemia / Treatment of Philadelphia chromosome-negative relapsed or refractory B-precursor acute lymphoblastic leukaemia (ALL) in patients 1 month and older.

Day 60 opinion

**Action:** For adoption

Oncology

### 2.3.10. Ibrutinib - Orphan - EMEA-001397-PIP03-14-M03

---

Janssen-Cilag International N.V.; Treatment of mature B-cell neoplasms / 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 60 opinion

**Action:** For adoption

Oncology

### 2.3.11. Inotuzumb ozogamicin - Orphan - EMEA-001429-PIP01-13-M02

---

Pfizer Ltd; Treatment of Acute Lymphoblastic Leukaemia / For the treatment of relapsed or refractory B cell precursor Acute Lymphoblastic Leukaemia

Day 60 opinion

**Action:** For adoption

Oncology / Haematology-Hemostaseology

### 2.3.12. Human Thrombin / Human Fibrinogen - EMEA-001149-PIP01-11-M04

---

Omrix Biopharmaceuticals N.V.; Treatment of cerebrospinal fluid leakage resulting from a surgical procedure, Treatment of haemorrhage resulting from a surgical procedure. / indicated for suture line sealing in dura mater closure, indicated for supportive treatment in surgery where standard surgical techniques are insufficient, for improvement of haemostasis., indicated for supportive treatment in surgery where standard surgical techniques are insufficient, for improvement of haemostasis.

Day 60 opinion

**Action:** For adoption

Other

### 2.3.13. Lanadelumab - Orphan - EMEA-001864-PIP01-15-M01

---

Shire Pharmaceuticals Ireland Limited; Treatment of hereditary angioedema

Day 60 opinion

**Action:** For adoption

Other

### 2.3.14. Sildenafil - Orphan - EMEA-000671-PIP01-09-M09

---

Pfizer Limited; Treatment of Pulmonary Arterial Hypertension (PAH)

Day 60 opinion

**Action:** For adoption

Other

### 2.3.15. Spheroids of human autologous matrix-associated chondrocytes - EMEA-001264-PIP01-12-M01

---

CO.DON AG; Treatment of symptomatic articular cartilage defects of the femoral condyle and the patella of the knee (International Cartilage Repair Society [ICRS] grade III or IV) with defect sizes up to 10 cm<sup>2</sup> / Treatment of symptomatic articular cartilage defects of the femoral condyle and the patella of the knee (International Cartilage Repair Society [ICRS] grade III or IV) with defect sizes up to 10 cm<sup>2</sup>

Day 60 opinion

**Action:** For adoption

Other

### 2.3.16. Aluminium hydroxide adsorbed, depigmented glutaraldehyde polymerised, allergen extract of Phleum pratense, Dactylis glomerata, Festuca elatior, Lolium perenne and Poa pratensis pollen (Grasses-Mix) (1/5) each - EMEA-000794-PIP01-09-M01

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LETI Pharma GmbH; J 30.1 Allergic rhinitis due to pollen, J 30.2 Other seasonal allergic rhinitis,, H10.1 Acute allergic conjunctivitis, J30.3 Other allergic rhinitis, J30.4 Allergic rhinitis, unspecified / Treatment of patients with allergic rhinitis with or without intermittent allergic asthma due to sensitisation against grass pollens (Gramineas family), Treatment of patients with allergic rhinoconjunctivitis with or without intermittent allergic asthma due to sensitisation against grass pollens (Gramineas family)

Day 60 opinion

**Action:** For adoption

Pneumology - Allergology

### 2.3.17. Aluminium hydroxide adsorbed, depigmented glutaraldehyde polymerised, allergen extract of Phleum pratense, Dactylis glomerata, Festuca elatior, Lolium

LETI Pharma GmbH; J 30.1 Allergic rhinitis due to pollen, J 30.2 Other seasonal allergic rhinitis,, H10.1 Acute allergic conjunctivitis, J30.3 Other allergic rhinitis, J30.4 Allergic rhinitis, unspecified / Treatment of patients with allergic rhinitis with or without intermittent allergic asthma due to sensitisation against grass pollens (Gramineas family), Treatment of patients with allergic rhinoconjunctivitis with or without intermittent allergic asthma due to sensitisation against grass pollens (Gramineas family)

Day 60 opinion

**Action:** For adoption

Pneumology - Allergology

#### 2.3.18. Aluminium hydroxide adsorbed, depigmented glutaraldehyde polymerised, allergic extract of Phleum pratense pollen - EMEA-000795-PIP01-09-M01

---

LETI Pharma GmbH; J 30.1 Allergic rhinitis due to pollen, J 30.2 Other seasonal allergic rhinitis,, H10.1 Acute allergic conjunctivitis, J30.3 Other allergic rhinitis, J30.4 Allergic rhinitis, unspecified / Treatment of patients with allergic rhinitis with or without intermittent allergic asthma due to sensitisation against grass pollens (Gramineas family), Treatment of patients with allergic rhinoconjunctivitis with or without intermittent allergic asthma due to sensitisation against grass pollens (Gramineas family)

Day 60 opinion

**Action:** For adoption

Pneumology - Allergology

### 2.4. Opinions on Re-examinations

#### 2.4.1. Crisaborole - EMEA-002065-PIP01-16

---

Pfizer Ltd; Mild to moderate atopic dermatitis

Day 30 opinion

**Action:** For adoption

Dermatology

### 2.5. Finalisation and adoption of opinions

## 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. Chloroprocaine Hydrochloride - EMEA-000639-PIP03-16

---

Peripheral nerve block (local anesthesia by perineural injection)

Day 90 discussion

**Action:** For discussion

Anaesthesiology

#### 3.1.2. Lucerastat - Orphan - EMEA-002095-PIP01-16

---

Actelion Registration Ltd.; Treatment of Fabry disease

Day 90 discussion

**Action:** For discussion

Endocrinology-Gynaecology-Fertility-Metabolism

#### 3.1.3. - EMEA-002109-PIP01-16

---

K75.8 Other specified inflammatory liver diseases (non-alcoholic steatohepatitis [NASH]) / Treatment of Non-Alcoholic Steatohepatitis (NASH) with mild to severe fibrosis (F1-F4) in paediatric subjects, 8 to < 18 years of age

Day 90 discussion

**Action:** For discussion

Gastroenterology-Hepatology

#### 3.1.4. Maralixibat Chloride - Orphan - EMEA-001475-PIP03-17

---

Shire Pharmaceuticals Ireland Limited; Treatment of Progressive Familial Intrahepatic Cholestasis

Day 90 discussion

**Action:** For discussion

Gastroenterology-Hepatology

#### 3.1.5. Glutamine (Levoglutamide) - Orphan - EMEA-001996-PIP02-16

---

Emmaus Medical Europe Ltd.; Sickle cell disease / Glutamine (Levoglutamide) is indicated for the prevention of sickle cell crises in adults and children older than 5 years suffering from Sickle Cell Disease.

Day 90 discussion

**Action:** For discussion

Haematology-Hemostaseology

### 3.1.6. Upadacitinib - EMEA-001741-PIP02-16

---

Treatment of Uclerative Colitis

Day 90 discussion

**Action:** For discussion

Immunology-Rheumatology-Transplantation

### 3.1.7. Obiltoxaximab - EMEA-002144-PIP01-17

---

Treatment of bacillary infection, Prevention of bacillary infection / Treatment of inhalation anthrax following exposure to Bacillus anthracis in combination with appropriate antibacterial drugs, Post-exposure prophylaxis of inhalation anthrax when alternative therapies are not available or are not appropriate.

Day 90 discussion

**Action:** For discussion

Infectious Diseases

### 3.1.8. Gilteritinib (as fumarate) - EMEA-002064-PIP01-16

---

Treatment of acute myeloid leukemia / Treatment of FLT3/ITD positive acute myeloid leukemia

Day 90 discussion

**Action:** For discussion

Oncology / Haematology-Hemostaseology

### 3.1.9. Recombinant Clostridium difficile Toxoid B / Recombinant Clostridium difficile Toxoid A - EMEA-002112-PIP01-16

---

Prevention of Clostridium difficile infection (CDI) / Active immunization for the prevention of primary Clostridium difficile infection in children and adolescents 2 to 18 years of age

Day 90 discussion

**Action:** For discussion

Vaccines

### 3.1.10. - EMEA-001527-PIP02-17

---

Treatment of obesity

Day 60 discussion

**Action:** For discussion

Endocrinology-Gynaecology-Fertility-Metabolism



### 3.1.11. Itacitinib - EMEA-002178-PIP01-17

---

Treatment of acute Graft versus Host Disease (D89.810, ICD-10-CM) / Treatment of steroid naïve paediatric population with acute graft versus host disease after allogeneic hematopoietic stem cell transplantation

Day 60 discussion

**Action:** For discussion

Immunology-Rheumatology-Transplantation

### 3.1.12. Fremanezumab - EMEA-001877-PIP03-17

---

Prevention of cluster headache

Day 60 discussion

**Action:** For discussion

Neurology

### 3.1.13. Setmelanotide - Orphan - EMEA-002209-PIP01-17

---

Rhythm Pharmaceuticals, Inc; Treatment of appetite and general nutrition disorders / Treatment of obesity and/or hyperphagia associated with genetic defects upstream of the MC4 receptor in the leptin-melanocortin pathway

Day 60 discussion

**Action:** For discussion

Nutrition

### 3.1.14. Afatinib - EMEA-001596-PIP02-17

---

Treatment of oropharyngeal, laryngeal or nasal epithelial carcinoma, Treatment of paediatric patients with tumours with known ErbB deregulations irrespective of tumour histology, Treatment of lung carcinoma, Treatment of urether and bladder carcinoma / Treatment of paediatric patients aged between  $\geq 1$  year and  $\leq 18$  years with recurrent or refractory tumours with known ErbB deregulation and irrespective of tumour histology.

Day 60 discussion

**Action:** For discussion

Oncology

### 3.1.15. Fosnetupitant / palonosetron - EMEA-001198-PIP03-17

---

Prevention of Chemotherapy-Induced Nausea and Vomiting

Day 60 discussion

**Action:** For discussion

Other

### 3.1.16. - EMEA-002160-PIP01-17

---

Prevention of human immunodeficiency virus (HIV-1) infection / Prevention of HIV-1 infection in children from 9 to less than 18 years of age

Day 60 discussion

**Action:** For discussion

Vaccines / Infectious Diseases

### 3.1.17. Clade C gp140 - EMEA-002221-PIP01-17

---

Prevention of human immunodeficiency virus (HIV-1) infection / Prevention of HIV-1 infection in children from 9 to less than 18 years of age

Day 60 discussion

**Action:** For discussion

Vaccines / Infectious Diseases

### 3.1.18. Mosaic gp140 - EMEA-002161-PIP01-17

---

Prevention of human immunodeficiency virus (HIV-1) infection / Prevention of HIV-1 infection in children from 9 to less than 18 years of age

Day 60 discussion

**Action:** For discussion

Vaccines / Infectious Diseases

### 3.1.19. Rosuvastatin calcium / Acetylsalicylic acid - EMEA-002239-PIP01-17

---

Prevention of cardiovascular events

Day 30 discussion

**Action:** For discussion

Cardiovascular Diseases

### 3.1.20. Baricitinib - EMEA-001220-PIP03-16

---

Treatment of atopic dermatitis / Treatment of patients with moderate to severe atopic dermatitis

Day 30 discussion

**Action:** For discussion

Dermatology

### 3.1.21. Fluorocholine (18F) - EMEA-002129-PIP02-17

---

Visualisation of choline metabolism in malignant neoplasms

Day 30 discussion

**Action:** For discussion

Diagnostic

### 3.1.22. Bis-choline tetrathiomolybdate - Orphan - EMEA-002232-PIP01-17

Wilson Therapeutics AB; Treatment of Wilson disease

Day 30 discussion

**Action:** For discussion

Endocrinology-Gynaecology-Fertility-Metabolism

### 3.1.23. Ethinyl estradiol / Dienogest - EMEA-002229-PIP01-17

Contraception / Oral contraception

Day 30 discussion

**Action:** For discussion

Endocrinology-Gynaecology-Fertility-Metabolism

### 3.1.24. Semaglutide - EMEA-001441-PIP03-17

Treatment of obesity

Day 30 discussion

**Action:** For discussion

Endocrinology-Gynaecology-Fertility-Metabolism

### 3.1.25. - Orphan - EMEA-002233-PIP01-17

Zealand Pharma A/S; Treatment of hypoglycaemia

Day 30 discussion

**Action:** For discussion

Endocrinology-Gynaecology-Fertility-Metabolism

### 3.1.26. Amiselimod - EMEA-002227-PIP01-17

Ulcerative colitis / Treatment of moderately to severely active UC in children and adolescents who have had an inadequate response with, lost response to, or were intolerant to either conventional therapy, a TNF-alpha antagonist or an integrin inhibitor.

Day 30 discussion

**Action:** For discussion

Gastroenterology-Hepatology

### 3.1.27. Ustekinumab - EMEA-000311-PIP05-17

---

Treatment of Ulcerative Colitis

Day 30 discussion

**Action:** For discussion

Gastroenterology-Hepatology

### 3.1.28. (6aR,10aR)-1-Hydroxy-6,6-dimethyl-3-(2-methyl-2-octanyl)-6a,7,10,10a-tetrahydro-6H-benzo[c]chromene-9-carboxylic acid - Orphan - EMEA-002069-PIP02-17

---

Corbus Pharmaceuticals Holdings Inc; Treatment of systemic sclerosis

Day 30 discussion

**Action:** For discussion

Immunology-Rheumatology-Transplantation

### 3.1.29. Riociguat - Orphan - EMEA-000718-PIP03-17

---

Bayer AG; Treatment of Systemic Sclerosis / Treatment of Diffuse Cutaneous Systemic Sclerosis (dcSSc)

Day 30 discussion

**Action:** For discussion

Immunology-Rheumatology-Transplantation

### 3.1.30. The whole range of unmanipulated autologous mononuclear cells derived from human umbilical cord blood (Hau-UCB-mnc) - Orphan - EMEA-001799-PIP02-17

---

BrainRepair UG (haftungsbeschränkt); Periventriculaleukomalacia (PVL) ICD-10-CM P91.2

Day 30 discussion

**Action:** For discussion

Neonatology - Paediatric Intensive Care

### 3.1.31. Vatiquinone - Orphan - EMEA-002235-PIP01-17

---

Edison Orphan Pharma BV; RARS2 syndrome (ICD10 code G31.9)

Day 30 discussion

**Action:** For discussion

Neurology

### 3.1.32. Tucatinib - EMEA-002242-PIP01-17

---

Treatment of breast malignant neoplasms

Day 30 discussion

**Action:** For discussion

Oncology

### 3.1.33. Recombinant human epidermal growth factor - EMEA-002258-PIP01-17

---

Diabetic foot ulcer

Day 30 discussion

**Action:** For discussion

Other / Endocrinology-Gynaecology-Fertility-Metabolism

### 3.1.34. Human donor hematopoietic stem and progenitor cells (HSPC) that have been treated ex vivo with Tat-MYC fusion protein - Orphan - EMEA-002185-PIP02-17

---

Taiga Biotechnologies, Inc.; Severe Combined Immunodeficiency

Day 30 discussion

**Action:** For discussion

Other / Immunology-Rheumatology-Transplantation

### 3.1.35. Purified Rabies virus - EMEA-002234-PIP01-17

---

Prevention of rabies disease, treatment of exposure to rabies virus

Day 30 discussion

**Action:** For discussion

Vaccines

## 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. Piperazine tetraphosphate / arteminol - EMEA-C-000153-PIP01-07-M04

---

Alfasigma S.p.A.; Treatment of uncomplicated malaria caused by Plasmodium falciparum

Day 30 discussion

**Action:** For discussion

Infectious Diseases

### 3.2.2. Clostridium Botulinum neurotoxin type A (150 kD) - EMEA-C-001039-PIP01-10-M02

---

Merz Pharmaceuticals GmbH; Treatment of muscle spasticity

Day 30 discussion

**Action:** For discussion

Neurology

3.2.3. Clostridium Botulinum neurotoxin type A (150 kD) -  
EMA-C1-001039-PIP02-12-M02

---

Merz Pharmaceuticals GmbH; Treatment of sialorrhea

Day 30 discussion

**Action:** For discussion

Neurology

3.2.4. Cytarabine (liposomal combination) / Daunorubicin (liposomal combination) -  
EMA-C1-001858-PIP02-16-M01

---

Jazz Pharmaceuticals Ireland Limited; Treatment of acute myeloid leukaemia

Day 30 discussion

**Action:** For discussion

Oncology

3.2.5. Recombinant human nerve growth factor - EMA-C-001729-PIP01-14-M01

---

Dompé farmaceutici SpA; Treatment of neurotrophic keratitis

Day 30 discussion

**Action:** For discussion

Ophthalmology

3.2.6. Influenza virus surface antigens (haemagglutinin and neuraminidase) of the  
following  
strains: A/<Official Strain>(H1N1), A/<Official Strain>(H3N2), B/<Official  
Strain>Yamagata lineage, B/<Official Strain>Victoria lineage -  
EMA-C1-001782-PIP01-15

---

Abbott Biologicals B.V.; Prevention of influenza infection

Day 30 discussion

**Action:** For discussion

Vaccines

3.2.7. Japanese-encephalitis virus, inactivated - EMA-C-000559-PIP01-09-M04

---

Valneva Austria GmbH; Prevention of Japanese encephalitis

Day 30 discussion

**Action:** For discussion

Vaccines

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

#### 3.3.1. Regadenoson - EMEA-000410-PIP01-08-M02

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Rapidscan Pharma Solutions EU Limited; Myocardial perfusion disturbances / Diagnostic evaluation of myocardial perfusion disturbances

Day 30 discussion

**Action:** For discussion

Cardiovascular Diseases

#### 3.3.2. Gadolinium,[α3,α6,α9-tris[[2-hydroxy-1-(hydroxymethyl)ethyl]amino]-3-oxopropyl]-3,6,9,15-tetraazabicyclo[9.3.1]pentadeca-1(15),11,13-triene-3,6,9-triacetato(3-)-κN3,κN6,κN9,κN15,κO3,κO6,κO9] - EMEA-001949-PIP01-16-M01

---

GUERBET; Detection and visualization of areas with disruption of the blood brain barrier and/or abnormal vascularity for the central nervous system (CNS), or of any type of diseases from different body regions (soft tissues, bone and internal body structures/organs) for diagnostic purposes.

Day 30 discussion

**Action:** For discussion

Diagnostic

#### 3.3.3. Empagliflozin - EMEA-000828-PIP01-09-M06

---

Boehringer Ingelheim International GmbH; Treatment of type 2 diabetes mellitus

Day 30 discussion

**Action:** For discussion

Endocrinology-Gynaecology-Fertility-Metabolism

#### 3.3.4. Linagliptin (as base) - EMEA-000498-PIP01-08-M07

---

Boehringer Ingelheim International GmbH; Type 2 Diabetes Mellitus

Day 30 discussion

**Action:** For discussion

Endocrinology-Gynaecology-Fertility-Metabolism

#### 3.3.5. Sitagliptin phosphate - EMEA-000470-PIP01-08-M10

---

Merck Sharp and Dohme (Europe), Inc.; Treatment of type 2 diabetes mellitus

Day 30 discussion

**Action:** For discussion

### 3.3.6. Baricitinib - EMEA-001220-PIP01-11-M02

---

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

Day 30 discussion

**Action:** For discussion

Immunology-Rheumatology-Transplantation

### 3.3.7. Human normal immunoglobulin - EMEA-001797-PIP01-15-M01

---

Octapharma Pharmazeutika Produktionsges.m.b.H; Primary Immunodeficiency Diseases

Day 30 discussion

**Action:** For discussion

Immunology-Rheumatology-Transplantation

### 3.3.8. Ixekizumab - EMEA-001050-PIP01-10-M03

---

Eli Lilly & Company Limited; Plaque psoriasis, Treatment of chronic idiopathic arthritis (including rheumatoid arthritis, psoriatic arthritis, ankylosing spondylarthritis and juvenile idiopathic arthritis) / Children with juvenile idiopathic arthritis subtypes of enthesitis-related arthritis (including JoAS) and juvenile psoriatic arthritis., Treatment of severe chronic plaque psoriasis in paediatric patients from the age of 6 years who are not adequately controlled by topical therapies.

Day 30 discussion

**Action:** For discussion

Immunology-Rheumatology-Transplantation

### 3.3.9. Tofacitinib - EMEA-000576-PIP01-09-M08

---

Pfizer Limited; Treatment of chronic idiopathic arthritis (including rheumatoid arthritis, psoriatic arthritis, ankylosing spondylitis and juvenile idiopathic arthritis)

Day 30 discussion

**Action:** For discussion

Immunology-Rheumatology-Transplantation

### 3.3.10. Ustekinumab - EMEA-000311-PIP03-11-M03

---

Janssen-Cilag International NV; Treatment of chronic idiopathic arthritis (including rheumatoid arthritis, spondyloarthritis, PsA and juvenile idiopathic arthritis [JIA])

Day 30 discussion

**Action:** For discussion



### 3.3.11. Ceftaroline fosamil - EMEA-000769-PIP01-09-M07

---

Pfizer Limited; Treatment of cSSTI (complicated skin and soft tissue infections) / Treatment of CAP (community-acquired pneumonia)

Day 30 discussion

**Action:** For discussion

Infectious Diseases

### 3.3.12. Cobicistat / atazanavir sulphate - EMEA-001465-PIP01-13-M02

---

Bristol-Myers Squibb Pharma EEIG; Treatment of HIV-1 infection / indicated in combination with other ARV medicinal products for the treatment of HIV-1 infected adults and children from 3 years of age without known mutations associated with resistance to atazanavir.

Day 30 discussion

**Action:** For discussion

Infectious Diseases

### 3.3.13. Oseltamivir phosphate - EMEA-000365-PIP01-08-M09

---

Roche Registration Limited; Treatment and prevention of influenza / Treatment and prevention of influenza in healthy and immunocompromised patients from 0 to less than 18 years of age

Day 30 discussion

**Action:** For discussion

Infectious Diseases

### 3.3.14. Posaconazole - EMEA-000468-PIP02-12-M04

---

Merck Sharp & Dohme (Europe), Inc.; Prevention of invasive fungal infections / For treatment of invasive fungal infections in the following paediatric patients: -Invasive aspergillosis in patients with disease that is refractory to amphotericin B or itraconazole or in patients who are intolerant of these medicinal products; , Treatment of invasive aspergillosis, -Patients receiving remission-induction chemotherapy for acute myelogenous leukemia (AML) or myelodysplastic syndromes (MDS) expected to result in prolonged neutropenia and who are at high risk of developing invasive fungal infections; - Hematopoietic stem cell transplant (HSCT) recipients who are undergoing high-dose immunosuppressive therapy for graft versus host disease and who are at high risk of developing invasive fungal infections.

Day 30 discussion

**Action:** For discussion

Infectious Diseases

### 3.3.15. Tedizolid phosphate - EMEA-001379-PIP01-12-M03

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Merck Sharp & Dohme (Europe) Inc.; Treatment of acute bacterial skin and skin structure infections / Treatment of acute bacterial skin and skin structure infections

Day 30 discussion

**Action:** For discussion

Infectious Diseases

### 3.3.16. Tenofovir alafenamide - EMEA-001584-PIP01-13-M03

---

Gilead Sciences International Ltd.; Treatment of chronic hepatitis B / indicated for the treatment of chronic hepatitis B infection in paediatric patients aged 2 years and above.

Day 30 discussion

**Action:** For discussion

Infectious Diseases

### 3.3.17. Lacosamide - EMEA-000402-PIP02-11-M05

---

UCB Pharma S.A.; Treatment of Epilepsy - Partial-onset seizures [G40.0 - G40.1 - G40.2]

Day 30 discussion

**Action:** For discussion

Neurology

### 3.3.18. Midostaurin - Orphan - EMEA-000780-PIP01-09-M04

---

Novartis Europharm Ltd; C92.0 Acute myeloid leukaemia, C94.3 Mast cell leukaemia, C96.2 Malignant mastocytosis / Treatment of paediatric patients with FLT3 mutated AML, newly diagnosed

Day 30 discussion

**Action:** For discussion

Oncology

### 3.3.19. Pembrolizumab - EMEA-001474-PIP02-16-M01

---

Merck Sharp & Dohme (Europe), Inc.; Treatment of all conditions included in the category of malignant neoplasms (except nervous system, haematopoietic and lymphoid tissue)., Treatment of Hodgkin Lymphoma / Treatment of advanced, untreated or previously treated, malignant melanoma in children from 12 year old to less than 18 years of age. Treatment as monotherapy of a PD-L1 positive paediatric malignant solid tumor in children from 6 months to less than 18 years of age., •Treatment of classical Hodgkin lymphoma with incomplete early response to front-linechemotherapy in children from 3 years to less than 18 years of age •Treatment of relapsed or refractory classical Hodgkin lymphoma in children from 5 years to less than 18 years of age

Day 30 discussion

**Action:** For discussion

Oncology

3.3.20. [Burosumab; Human recombinant IgG1 monoclonal antibody to fibroblast growth factor 23 \(FGF23\); KRN23 - Orphan - EMEA-001659-PIP01-15-M03](#)

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Ultragenyx Pharmaceutical Inc.; X-linked Hypophosphatemia

Day 30 discussion

**Action:** For discussion

Other

3.3.21. [Conestat alfa - EMEA-000367-PIP01-08-M07](#)

---

Pharming Group N.V.; D84.1 Defects in the complement system C1 esterase inhibitor (C1-INH) deficiency / Treatment of acute attacks of angioedema associated with hereditary C1 esterase inhibitor deficiency

Day 30 discussion

**Action:** For discussion

Other

3.3.22. [Ivacaftor / lumacaftor - EMEA-001582-PIP01-13-M07](#)

---

Vertex Pharmaceuticals (Europe) Limited; Treatment of cystic fibrosis

Day 30 discussion

**Action:** For discussion

Other

3.3.23. [Matrix applied characterised autologous cultured chondrocytes - EMEA-000979-PIP01-10-M02](#)

---

Vericel Denmark ApS; repair of symptomatic, full-thickness cartilage defects of the knee /

Day 30 discussion

**Action:** For discussion

Other

3.3.24. [Birch pollen extract \(Betula verrucosa\) - EMEA-001879-PIP01-15-M01](#)

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ALK Abelló A/S; J30.1 Allergic rhinitis due to pollen / Treatment of tree pollen allergic rhinitis and/or conjunctivitis

Day 30 discussion

**Action:** For discussion

Pneumology - Allergology

### 3.3.25. Reslizumab - EMEA-001202-PIP02-13-M02

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Teva Pharmaceuticals Europe; Treatment of asthma / indicated as add-on treatment in adult patients with severe eosinophilic asthma

Day 30 discussion

**Action:** For discussion

Pneumology - Allergology

### 3.3.26. N-[(1,3-dicyclohexyl-6-hydroxy-2,4-dioxo-1,2,3,4-tetrahydro-5-pyrimidinyl)carbonyl]glycine - EMEA-001452-PIP01-13-M01

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GlaxoSmithKline R & D; Treatment of anaemia associated with chronic renal disease

Day 30 discussion

**Action:** For discussion

Uro-nephrology / Haematology-Hemostaseology

### 3.3.27. Outer Membrane Vesicles (OMV) from Neisseria Meningitidis serogroup B strain NZ98/254 measured as amount of total protein containing the PorA P1.4 / Recombinant Neisseria Meningitidis serogroup B fHbp fusion protein / Recombinant Neisseria Meningitidis serogroup B NadA protein / Recombinant Neisseria Meningitidis serogroup B NHBA fusion protein - EMEA-000139-PIP01-07-M02

---

GSK Vaccines S.r.l.; Prevention of meningitis

Day 30 discussion

**Action:** For discussion

Vaccines

## 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 DD February 2018 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

None

## 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

None

## 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. User manual on CxMP/EMA external representation

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**Action:** For information

### 9.2. Coordination with EMA Scientific Committees or CMDh-v

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

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**Action:** For information

Joint CHMP/PDCO session (TBC)

**Action:** For discussion

9.2.2. CHMP Oncology Working Party Workshop on: Histology – independent indications in Oncology

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PDCO member: Koenraad Norga

**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. Non-clinical Working Group: Call for additional members

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**Action:** For information

9.3.3. Formulation Working Group

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PDCO member: Brian Aylward

**Action:** For information

9.3.4. Formulation Working Group: PDCO FWG participants 2018

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PDCO member: Brian Aylward

**Action:** For adoption

9.3.5. Guideline on the clinical evaluation of vaccines

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Revised draft for comments by 14 November 2017

**Action:** For discussion

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

9.4.2. European Commission

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10 Year report of the Paediatric regulation

**Action:** For information

9.5. **Cooperation with International Regulators**

None

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

None

## 9.7. PDCO work plan

### 9.7.1. Draft PDCO Work plan 2018

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**Action:** For discussion

## 9.8. Planning and reporting

### 9.8.1. Report on the PDCO Strategic Review & Learning meeting in Tallinn, 4-6 October 2017

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PDCO member: Irja Lutsar

**Action:** For information

## 10. Any other business

### 10.1. AOB topic

None

## 11. Breakout sessions

### 11.1.1. Paediatric oncology

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**Action:** For discussion on Thursday, 14:00 - 15:00, room 3M

### 11.1.2. Neonatology

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**Action:** For discussion on Thursday, 14:00 - 15:00, room 3L

### 11.1.3. Inventory

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**Action:** For discussion on Thursday, 14:00 - 15:00, delegates' lounge

## 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/)