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SCIENCE MEDICINES HEALTH

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Inspections, Human Medicines Pharmacovigilance and Committees Division

## Paediatric Committee (PDCO)

Draft agenda for the meeting on 24-27 July 2018

Chair: Dirk Mentzer – Vice-Chair: Koenraad Norga

24 July 2018, 14:00 - 19:30, room 3A

25 July 2018, 08:30 - 19:30, room 3A

26 July 2018, 08:30 - 19:00, room 3A

27 July 2018, 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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## 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 24-27 July 2018. See July 2018 PDCO minutes (to be published post August 2018 PDCO meeting).

### 1.2. Adoption of agenda

PDCO agenda for 24-27 July 2018.

### 1.3. Adoption of the minutes

PDCO minutes for 26-29 June 2018.

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

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

Day 120 opinion

**Action:** For adoption

Dermatology

#### 2.1.2. EMEA-002208-PIP01-17

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Treatment of psoriasis, Treatment of Crohn's disease, Treatment of ulcerative colitis / Treatment of moderate to severely active Crohn's disease in paediatric patients aged 2 to less than 18 years of age, Treatment of moderate to severely active ulcerative colitis in paediatric patients aged 2 to less than 18 years of age, Treatment of moderate-to-severe plaque psoriasis in paediatric patients aged 6 to less than 18 years of age.

Day 120 opinion

**Action:** For adoption

Dermatology / Gastroenterology-Hepatology

### 2.1.3. Inclisiran sodium - EMEA-002214-PIP01-17

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Treatment of familial hypercholesterolaemia / Inclisiran is indicated to lower LDL-C in adults and children aged 8 years old and older with heterozygous familial hypercholesterolemia in combination with other lipid lowering therapies, Inclisiran is indicated to lower LDL-C in adults and children aged 8 years old and older with homozygous familial hypercholesterolemia in combination with other lipid lowering therapies.

Day 120 opinion

**Action:** For adoption

Endocrinology-Gynaecology-Fertility-Metabolism

### 2.1.4. Anti-Mucosal Addressin Cell Adhesion Molecule Antibody - EMEA-002218-PIP01-17

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Treatment of Ulcerative Colitis, Treatment of Crohn's Disease / Treatment of moderate to severe active Crohn's Disease, Treatment of moderate to severe active Ulcerative Colitis

Day 120 opinion

**Action:** For adoption

Gastroenterology-Hepatology

### 2.1.5. Human donor haematopoietic stem and progenitor cells that have been treated ex vivo with the protein transduction domain of the HIV-1 transactivation protein fused to MYC transcription factor - Orphan - EMEA-002185-PIP02-17

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Taiga Biotechnologies, Inc; Severe combined immunodeficiency

Day 120 opinion

**Action:** For adoption

Immunology-Rheumatology-Transplantation

### 2.1.6. Ibalizumab - EMEA-002311-PIP01-17

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Treatment of human immunodeficiency virus (HIV-1) infection / Ibalizumab, a CD4 domain 2-directed HIV-1 inhibitor, in combination with other antiretroviral(s), is indicated for the treatment of children and adolescents (aged 6 to less than 18 years) infected with HIV-1 resistant to at least 1 agent in 3 different classes.

Day 120 opinion

**Action:** For adoption

Infectious Diseases

#### 2.1.7. Sarizotan hydrochloride - Orphan - EMEA-001808-PIP03-17

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Newron Pharmaceuticals SpA; Treatment of Rett syndrome

Day 120 opinion

**Action:** For adoption

Neurology

#### 2.1.8. Survival Motor Neuron Gene by Self-Complementary Adeno Associated Virus Serotype 9 (AAV9) - Orphan - EMEA-002168-PIP01-17

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AveXis EU Ltd; Treatment of spinal muscular atrophy Type 1

Day 120 opinion

**Action:** For adoption

Neurology

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

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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 120 opinion

**Action:** For adoption

Oncology

#### 2.1.10. Entrectinib - EMEA-002096-PIP01-16

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Treatment of all conditions included in the category of malignant neoplasms (except central nervous system tumours, haematopoietic and lymphoid tissue neoplasms) / For the treatment of paediatric patients with NTRK fusion-positive solid tumours

Day 120 opinion

**Action:** For adoption

Oncology

#### 2.1.11. Ivosidenib - Orphan - EMEA-002247-PIP03-17

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Agios Pharmaceuticals, Inc.; Treatment of Acute Myeloid Leukaemia / Treatment of paediatric patients from 2 to less than 18 years of age with newly diagnosed and relapsed or refractory (R/R) AML with an isocitrate dehydrogenase-1 (IDH1) mutation.

Day 120 opinion

**Action:** For adoption

Oncology

#### 2.1.12. Sulindac / Eflornithine - Orphan - EMEA-001518-PIP02-16

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Cancer Prevention Pharma Ltd.; Treatment of Familial Adenomatous Polyposis

Day 120 opinion

**Action:** For adoption

Oncology

#### 2.1.13. Sodium thiosulfate - EMEA-002147-PIP02-17

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Prevention of platinum-induced ototoxic hearing loss / Prevention of ototoxicity in patients > 1 month and <18 years of age receiving platinum-based chemotherapy for localised tumours

Day 120 opinion

**Action:** For adoption

Oncology / Oto-rhino-laryngology

#### 2.1.14. Autologous cartilage derived cultured chondrocytes - EMEA-002217-PIP01-17

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Treatment of cartilage disorders

Day 120 opinion

**Action:** For adoption

Other

#### 2.1.15. Palovarotene - EMEA-001662-PIP03-17

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Treatment of Multiple Osteochondromas (MO)

Day 120 opinion

**Action:** For adoption

Other

#### 2.1.16. Recombinant human acid ceramidase - Orphan - EMEA-002266-PIP01-17

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Enzyvant Farber Ireland Ltd; Farber disease

Day 120 opinion

**Action:** For adoption

Other

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

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Faron Pharmaceuticals Ltd; Treatment of Acute Respiratory Distress Syndrome

Day 120 opinion

**Action:** For adoption

Pneumology - Allergology

#### 2.1.18. Ad26.RSV.preF - EMEA-002172-PIP02-17

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Prevention of lower respiratory tract disease caused by respiratory syncytial virus (RSV)

Day 120 opinion

**Action:** For adoption

Vaccines / Infectious Diseases

#### 2.1.19. Indapamide hemihydrate / perindopril tert-butylamine / rosuvastatin calcium / acetylsalicylic acid - EMEA-002366-PIP01-18

---

Treatment of cardiovascular disease / For the secondary prevention of cardiovascular accidents as substitution therapy in patients adequately controlled with the mono-components given concomitantly at equivalent therapeutic doses.

Day 60 opinion

**Action:** For adoption

Cardiovascular Diseases

#### 2.1.20. Rosuvastatin Calcium / Omega-3-acid ethyl esters 90 - EMEA-002384-PIP01-18

---

ICD10: E78.2

Day 60 opinion

**Action:** For adoption

Cardiovascular Diseases

#### 2.1.21. Arimoclomol citrate - Orphan - EMEA-001748-PIP02-18

---

Orphazyme A/S; Treatment of amyotrophic lateral sclerosis, Treatment of sporadic inclusion body myositis

Day 60 opinion

**Action:** For adoption

Neurology

#### 2.1.22. Elotuzumab - EMEA-002377-PIP01-18

---

Treatment of multiple myeloma

Day 60 opinion

**Action:** For adoption

Oncology

#### 2.1.23. Nadofaragene firadenovec - EMEA-002376-PIP01-18

---

Mesothelioma

Day 60 opinion

**Action:** For adoption

Oncology

#### 2.1.24. Tepotinib - EMEA-002345-PIP01-18

---

Treatment of lung malignant neoplasms

Day 60 opinion

**Action:** For adoption

Oncology

#### 2.1.25. Dexamethasone / Levofloxacin - EMEA-002375-PIP01-18

---

Prevention and treatment of inflammation and prevention of infection associated with cataract surgery

Day 60 opinion

**Action:** For adoption

Ophthalmology

#### 2.1.26. Fasinumab - EMEA-002059-PIP01-16

---

Chronic pain

Day 60 opinion

**Action:** For adoption

Pain

## 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. Autologous CD34+ haematopoietic stem cells transduced with lentiviral vector encoding the human betaA-T87Q-globin gene - EMEA-C1-001665-PIP01-14-M02

---

bluebird bio (Germany) GmbH; Treatment of  $\beta$ -thalassaemia

Day 60 letter

**Action:** For adoption

Haematology-Hemostaseology

#### 2.2.2. Upadacitinib - EMEA-C1-001741-PIP01-14-M01

---

AbbVie Ltd; Treatment of Chronic Idiopathic Arthritis (including rheumatoid arthritis, psoriatic arthritis, spondyloarthritis and juvenile idiopathic arthritis)

Day 60 letter

**Action:** For adoption

Immunology-Rheumatology-Transplantation

#### 2.2.3. Fenfluramine hydrochloride - EMEA-C2-001990-PIP01-16

---

Zogenix International Ltd; Treatment of Dravet syndrome

Day 60 letter

**Action:** For adoption

Neurology

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

#### 2.3.1. Gadolinium - EMEA-001949-PIP01-16-M02

---

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 60 opinion

**Action:** For adoption

Diagnostic

### 2.3.2. Dapagliflozin - EMEA-000694-PIP01-09-M07

---

AstraZeneca AB; Treatment of Type 2 Diabetes

Day 60 opinion

**Action:** For adoption

Endocrinology-Gynaecology-Fertility-Metabolism

### 2.3.3. Ertugliflozin L-PGA - EMEA-001533-PIP01-13-M01

---

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

Day 60 opinion

**Action:** For adoption

Endocrinology-Gynaecology-Fertility-Metabolism

### 2.3.4. Exenatide - EMEA-000689-PIP01-09-M08

---

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

Day 60 opinion

**Action:** For adoption

Endocrinology-Gynaecology-Fertility-Metabolism

### 2.3.5. Recombinant Human alpha-galactosidase A - Orphan - EMEA-001828-PIP01-15-M01

---

Protalix Ltd; Treatment of Fabry disease

Day 60 opinion

**Action:** For adoption

Endocrinology-Gynaecology-Fertility-Metabolism

2.3.6. Polyethylene Glycol 3350 / Potassium Chloride / Sodium Chloride / Ascorbic Acid / Sodium Ascorbate / Sodium Sulfate - EMEA-001705-PIP02-15-M01

---

Norgine Limited; Bowel cleansing prior to clinical procedures

Day 60 opinion

**Action:** For adoption

Gastroenterology-Hepatology

2.3.7. Tofacitinib - EMEA-000576-PIP03-12-M01

---

Pfizer Limited; Ulcerative colitis / Treatment of children and adolescents aged 2 to <18 years of age with moderate to severe ulcerative colitis, who have had an inadequate response, lost response, or were intolerant to either conventional therapy or a biologic agent.

Day 60 opinion

**Action:** For adoption

Gastroenterology-Hepatology

2.3.8. Caplacizumab (anti-von Willebrand Factor Nanobody) - Orphan - EMEA-001157-PIP01-11-M02

---

Abylnx NV; Treatment of acquired thrombotic thrombocytopenic purpura

Day 60 opinion

**Action:** For adoption

Haematology-Hemostaseology

2.3.9. Eftrenonacog alfa - Orphan - EMEA-000914-PIP01-10-M04

---

Swedish Orphan Biovitrum AB (publ); Hereditary Factor IX Deficiency - D67

Day 60 opinion

**Action:** For adoption

Haematology-Hemostaseology

2.3.10. Human fibrinogen concentrate - EMEA-001931-PIP01-16-M01

---

Biotest AG; Treatment of congenital fibrinogen deficiency

Day 60 opinion

**Action:** For adoption

Haematology-Hemostaseology

### 2.3.11. Belimumab - EMEA-000520-PIP02-13-M02

---

Glaxo Group Limited; Treatment of systemic lupus erythematosus

Day 60 opinion

**Action:** For adoption

Immunology-Rheumatology-Transplantation

### 2.3.12. Emapalumab - Orphan - EMEA-002031-PIP01-16-M02

---

Novimmune B.V; Treatment of Haemophagocytic Lymphohistiocytosis

Day 60 opinion

**Action:** For adoption

Immunology-Rheumatology-Transplantation

### 2.3.13. Isavuconazonium (sulfate) - Orphan - EMEA-001301-PIP02-12-M02

---

Basilea Pharmaceutica International Ltd.; Treatment of mucormycosis, Treatment of invasive aspergillosis

Day 60 opinion

**Action:** For adoption

Infectious Diseases

### 2.3.14. Rilpivirine (as hydrochloride) - EMEA-000317-PIP01-08-M10

---

Janssen-Cilag International NV; Treatment of human immunodeficiency virus (HIV-1) infection / Rilpivirine is indicated in combination with other antiretroviral (ARV) medicinal products, for the treatment of human immunodeficiency virus (HIV-1) infection in ARV-naïve paediatric patients from 2 to less than 18 years with a baseline viral load below 100,000 HIV-1 RNA copies/mL.

Day 60 opinion

**Action:** For adoption

Infectious Diseases

### 2.3.15. Simeprevir - EMEA-000625-PIP01-09-M03

---

Janssen-Cilag International NV; Treatment of Chronic Viral Hepatitis C (HCV) / Treatment of chronic hepatitis C genotype 1 and genotype 4 infection in pediatric patients aged 3 to less than 18 years.

Day 60 opinion

**Action:** For adoption

Infectious Diseases

2.3.16. [Tenofovir alafenamide / emtricitabine / cobicistat / darunavir - EMEA-001825-PIP01-15-M02](#)

---

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

Day 60 opinion

**Action:** For adoption

Infectious Diseases

2.3.17. [Tenofovir Alafenamide / Emtricitabine / Cobicistat / Elvitegravir - EMEA-001460-PIP01-13-M03](#)

---

Gilead Sciences International Ltd.; Treatment of human immunodeficiency virus (HIV-1) infection / Indicated for the treatment of HIV-1 infection in paediatric patients from 6 years to less than 18 years.

Day 60 opinion

**Action:** For adoption

Infectious Diseases

2.3.18. [Tenofovir disoproxil / rilpivirine / emtricitabine - EMEA-000774-PIP01-09-M03](#)

---

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

Day 60 opinion

**Action:** For adoption

Infectious Diseases

2.3.19. [Autologous haematopoietic stem cells transduced with lentiviral vector Lenti-D encoding the human ATP-binding cassette, sub-family D \(ALD\), member 1 \(ABCD1\) from cDNA - Orphan - EMEA-001244-PIP01-11-M02](#)

---

bluebird bio France; Adrenoleukodystrophy

Day 60 opinion

**Action:** For adoption

Neurology

2.3.20. [Perampanel - EMEA-000467-PIP01-08-M10](#)

---

Eisai Europe Limited; Treatment of treatment-resistant epilepsies / Adjunctive therapy in

patients with other paediatric epilepsies, Adjunctive therapy in patients with refractory partial onset seizures including secondarily generalised seizures

Day 60 opinion

**Action:** For adoption

Neurology

#### 2.3.21. Retigabine - EMEA-000116-PIP01-07-M09

---

Glaxo Group Limited; Treatment of Lennox-Gastaut Syndrome, Treatment of epilepsy with partial onset seizures

Day 60 opinion

**Action:** For adoption

Neurology

#### 2.3.22. Ponatinib (as hydrochloride) - Orphan - EMEA-001186-PIP01-11-M02

---

Incyte Biosciences UK Ltd.; Chronic myeloid leukaemia, Philadelphia chromosome positive acute lymphoblastic leukaemia / Treatment of the paediatric population with Ph+ ALL who are resistant or intolerant to prior TKI therapy, or who have the T315I mutation., Treatment of the paediatric population with chronic (CP), accelerated (AP), or blast phase (BP) CML who are resistant or intolerant to prior tyrosine kinase inhibitor (TKI) therapy, or who have the T315I mutation.

Day 60 opinion

**Action:** For adoption

Oncology

#### 2.3.23. Quizartinib - Orphan - EMEA-001821-PIP01-15-M02

---

Daiichi Sankyo Europe GmbH; Treatment of acute myeloid leukaemia / For the treatment of paediatric patients aged from 1 month to less than 18 years of age with newly diagnosed AML with FLT3-ITD mutations., For the treatment of paediatric patients aged from 1 month to less than 18 years of age with refractory or relapsed AML with FLT3-ITD mutations after failure of front line intensive chemotherapy regimen, in combination with standard chemotherapy.

Day 60 opinion

**Action:** For adoption

Oncology

#### 2.3.24. Eliglustat - Orphan - EMEA-000461-PIP02-11-M03

---

Genzyme Europe B.V.; Treatment of Gaucher Disease Type 1, Treatment of Gaucher Disease Type 3

Day 60 opinion

**Action:** For adoption

Other

#### 2.3.25. Vamorolone - Orphan - EMEA-001794-PIP02-16-M01

---

ReveraGen BioPharma Ltd; Treatment of Duchenne muscular dystrophy

Day 60 opinion

**Action:** For adoption

Other

#### 2.3.26. Dupilumab - EMEA-001501-PIP02-13-M03

---

sanofi-aventis recherche & développement; Asthma

Day 60 opinion

**Action:** For adoption

Pneumology - Allergology

#### 2.3.27. Mepolizumab - Orphan - EMEA-000069-PIP04-13-M02

---

GSK Trading Services Limited; Vasculitides / Treatment of paediatric patients aged 6 to 17 years with eosinophilic granulomatosis with polyangiitis (EGPA) using corticosteroid therapy with or without concomitant immunosuppressant therapy.

Day 60 opinion

**Action:** For adoption

Pneumology - Allergology

#### 2.3.28. Mometasone (furoate) / Indacaterol (acetate) - EMEA-001217-PIP01-11-M05

---

Novartis Europharm Limited; Treatment of asthma

Day 60 opinion

**Action:** For adoption

Pneumology - Allergology

### 2.3.29. Brexpiprazole - EMEA-001185-PIP01-11-M05

---

Otsuka Europe Development and Commercialisation Limited, Zweigniederlassung, Frankfurt am Main; Treatment of schizophrenia in adolescents 13 to 17 years of age

Day 60 opinion

**Action:** For adoption

Psychiatry

### 2.3.30. Cariprazine hydrochloride - EMEA-001652-PIP01-14-M02

---

Gedeon Richter Plc.; F20 Schizophrenia

Day 60 opinion

**Action:** For adoption

Psychiatry

### 2.3.31. Loxapine - EMEA-001115-PIP01-10-M06

---

Ferrer Internacional, S.A.; Bipolar disorder, Schizophrenia / For rapid control of agitation in patients with schizophrenia, For rapid control of agitation in patients with bipolar disorder

Day 60 opinion

**Action:** For adoption

Psychiatry

### 2.3.32. Lanthanum carbonate hydrate - EMEA-000637-PIP02-10-M06

---

Shire Pharmaceutical Contracts Ltd; Hyperphosphataemia

Day 60 opinion

**Action:** For adoption

Uro-nephrology

## 2.4. Opinions on Re-examinations

No items.

## 2.5. Opinions on Review of Granted Waivers

None

## 2.6. Finalisation and adoption of opinions

## 2.7. Partial Compliance Checks completed by EMA

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

None

## 2.8. Revision of the PDCO opinions

### 2.8.1. Landiolol hydrochloride - EMEA-001150-PIP02-13-M02

---

AOP Orphan Pharmaceuticals AG; Treatment of supraventricular arrhythmias

Day 60 opinion

**Action:** For adoption

Cardiovascular Diseases

## 3. Discussion of applications

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

### 3.1. Discussions on Products D90-D60-D30

#### 3.1.1. EMEA-002350-PIP01-18

---

Treatment of psoriasis / Treatment of moderate to severe plaque psoriasis in paediatric patients 6 years of age and older

Day 60 discussion

**Action:** For discussion

Dermatology

#### 3.1.2. Chemically modified recombinant human sulfamidase - Orphan - EMEA-002380-PIP01-18

---

Swedish Orphan Biovitrum AB (publ); Mucopolysaccharidosis type IIIA (MPS IIIA)

Day 60 discussion

**Action:** For discussion

Endocrinology-Gynaecology-Fertility-Metabolism

### 3.1.3. Asciminib - EMEA-002347-PIP01-18

---

Treatment of Philadelphia positive Chronic Myelogenous Leukemia in chronic phase

Day 60 discussion

**Action:** For discussion

Haematology-Hemostaseology

### 3.1.4. Concizumab - Orphan - EMEA-002326-PIP03-18

---

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

Day 60 discussion

**Action:** For discussion

Haematology-Hemostaseology

### 3.1.5. Anti-IL-21 humanized immunoglobulin G1-kappa monoclonal antibody - EMEA-002374-PIP01-18

---

Treatment of Systemic Lupus Erythematosus (SLE)

Day 60 discussion

**Action:** For discussion

Immunology-Rheumatology-Transplantation

### 3.1.6. Fenebrutinib - EMEA-002349-PIP01-18

---

Chronic idiopathic arthritis (including RA, axial spondyloarthritis, PsA, and JIA) / Treatment of active JIA (i.e., seropositive [RF positive] polyarthritis, seronegative [RF negative] polyarthritis, enthesitis related arthritis, psoriatic arthritis, persistent sJIA without systemic features, oligoarthritis [persistent and extended], and undifferentiated arthritis) in patients 2 years of age to less than 18 years of age

Day 60 discussion

**Action:** For discussion

Immunology-Rheumatology-Transplantation

### 3.1.7. Guselkumab - EMEA-001523-PIP03-18

---

Treatment of chronic idiopathic arthritis (including rheumatoid arthritis, spondyloarthritis, PsA and juvenile idiopathic arthritis [JIA])

Day 60 discussion

**Action:** For discussion

Immunology-Rheumatology-Transplantation

### 3.1.8. Rilpivirine (as free base) - EMEA-000317-PIP02-18

---

Treatment of human immunodeficiency virus (HIV-1) infection / In combination with cabotegravir long acting, treatment of HIV-1 infection in pediatric patients from 6 to less than 18 years of age who are virologically suppressed (HIV-1 RNA <50 copies/mL) and no known or suspected resistance to either rilpivirine or cabotegravir

Day 60 discussion

**Action:** For discussion

Infectious Diseases

### 3.1.9. Suvratumab (anti-Staphylococcus aureus alpha toxin monoclonal antibody) - EMEA-002337-PIP01-18

---

Prevention of nosocomial pneumonia caused by Staphylococcus aureus

Day 60 discussion

**Action:** For discussion

Infectious Diseases

### 3.1.10. Avapritinib - Orphan - EMEA-002358-PIP02-18

---

Blueprint Medicines Corporation; Treatment of all conditions included in the category of malignant neoplasms (except haematopoietic and lymphoid tissue neoplasms) / Treatment of paediatric patients with advanced solid tumors harboring mutations in either KIT or PDGFR $\alpha$ .

Day 60 discussion

**Action:** For discussion

Oncology

### 3.1.11. Spartalizumab - EMEA-002351-PIP01-18

---

Treatment of melanoma / Treatment of adolescent patients with melanoma containing BRAF V600 activating mutations

Day 60 discussion

**Action:** For discussion

Oncology

### 3.1.12. Odiparcil - Orphan - EMEA-002256-PIP01-17

---

Inventiva SA; Treatment of mucopolysaccharidosis type VI (Maroteaux-Lamy syndrome)

Day 60 discussion

**Action:** For discussion

Other

### 3.1.13. (R)-azasetron (as besylate) - Orphan - EMEA-002165-PIP02-18

---

Sensorion SA; Ototoxicity, poisoning due to cisplatin, Sudden Sensorineural Hearing Loss / Treatment of Sudden Sensorineural Hearing Loss, Prevention of cisplatin-induced ototoxicity

Day 60 discussion

**Action:** For discussion

Oto-rhino-laryngology

### 3.1.14. EMEA-002324-PIP01-17

---

Treatment of Cystic Fibrosis

Day 60 discussion

**Action:** For discussion

Pneumology - Allergology

### 3.1.15. EMEA-002191-PIP02-17

---

Treatment of Cystic Fibrosis

Day 60 discussion

**Action:** For discussion

Pneumology - Allergology

### 3.1.16. Ad26.ZEBOV - EMEA-002307-PIP01-17

---

Prevention of Ebola Virus Disease / Prevention of EVD in children aged  $\geq 1$  year

Day 60 discussion

**Action:** For discussion

Vaccines / Infectious Diseases

### 3.1.17. [MVA-BN-Filo - EMEA-002308-PIP01-17](#)

---

Prevention of Ebola Virus Disease / Prevention of EVD in children aged  $\geq 1$  year

Day 60 discussion

**Action:** For discussion

Vaccines / Infectious Diseases

### 3.1.18. [Indapamide / Perindopril arginine / Atorvastatin calcium trihydrate - EMEA-002395-PIP01-18](#)

---

Treatment of Essential Hypertension, Treatment of Cardiovascular diseases, Prevention of Cardiovascular diseases, Treatment of Elevated Cholesterol

Day 30 discussion

**Action:** For discussion

Cardiovascular Diseases

### 3.1.19. [EMEA-002378-PIP01-18](#)

---

Treatment of acute heart failure

Day 30 discussion

**Action:** For discussion

Cardiovascular Diseases

### 3.1.20. [EMEA-002363-PIP01-18](#)

---

Treatment of transthyretin-mediated amyloidosis (ATTR amyloidosis) - cardiomyopathy amyloidosis, Treatment of transthyretin-mediated amyloidosis (ATTR amyloidosis) - polyneuropathy amyloidosis

Day 30 discussion

**Action:** For discussion

Cardiovascular Diseases / Neurology

### 3.1.21. [Dihomo- \$\gamma\$ -linolenic acid \(DGLA\) - EMEA-002364-PIP01-18](#)

---

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

Day 30 discussion

**Action:** For discussion

Dermatology

### 3.1.22. Dihomo- $\gamma$ -linolenic acid (DGLA) - EMEA-002364-PIP02-18

---

Treatment of atopic dermatitis / Treatment of pruritus associated with mild to moderate atopic dermatitis, Treatment of mild to moderate atopic dermatitis

Day 30 discussion

**Action:** For discussion

Dermatology

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

---

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

Day 30 discussion

**Action:** For discussion

Endocrinology-Gynaecology-Fertility-Metabolism

### 3.1.24. Tirzepatide - EMEA-002360-PIP01-18

---

Treatment of Type 2 diabetes mellitus

Day 30 discussion

**Action:** For discussion

Endocrinology-Gynaecology-Fertility-Metabolism

### 3.1.25. Cell-free solution of lysed Escherichia coli culture, strain Laves - EMEA-002393-PIP01-18

---

Treatment of irritable bowel syndrome, treatment of colitis (excluding infective)

Day 30 discussion

**Action:** For discussion

Gastroenterology-Hepatology

### 3.1.26. Ianalumab - EMEA-002338-PIP02-18

---

Primary Sjögren's Syndrome (pSS)

Day 30 discussion

**Action:** For discussion

Immunology-Rheumatology-Transplantation

### 3.1.27. Sarilumab - EMEA-001045-PIP02-18

---

Vasculitides

Day 30 discussion

**Action:** For discussion

Immunology-Rheumatology-Transplantation

### 3.1.28. Iclaprim mesylate - EMEA-002391-PIP01-18

---

Infection with Gram-positive bacteria / Treatment of acute bacterial skin and skin structure infections caused by susceptible strains of Gram-positive bacteria

Day 30 discussion

**Action:** For discussion

Infectious Diseases

### 3.1.29. Oteseconazole - EMEA-002392-PIP01-18

---

Treatment of vulvovaginal candidiasis

Day 30 discussion

**Action:** For discussion

Infectious Diseases

### 3.1.30. EMEA-002386-PIP01-18

---

Duchenne Muscular Dystrophy (DMD) / Treatment of Duchenne Muscular Dystrophy (DMD) in ambulant paediatric patients from 5 years old

Day 30 discussion

**Action:** For discussion

Neurology

### 3.1.31. A synthetic ribonucleic acid oligonucleotide directed against superoxide dismutase 1 (SOD1) messenger ribonucleic acid - Orphan - EMEA-002403-PIP01-18

---

Biogen Idec Ltd; Treatment of Amyotrophic lateral sclerosis

Day 30 discussion

**Action:** For discussion

Neurology

### 3.1.32. [Anti-alpha synuclein monoclonal antibody - EMEA-002367-PIP01-18](#)

---

Treatment of Parkinson's Disease

Day 30 discussion

**Action:** For discussion

Neurology

### 3.1.33. [Ofatumumab - EMEA-002397-PIP01-18](#)

---

Treatment of Multiple Sclerosis / Treatment of relapsing remitting multiple sclerosis

Day 30 discussion

**Action:** For discussion

Neurology

### 3.1.34. [EMEA-002318-PIP01-18](#)

---

Treatment of melanoma

Day 30 discussion

**Action:** For discussion

Oncology

### 3.1.35. [Anti-FGFR2b humanised IgG1 monoclonal antibody \(bemarituzumab\) - EMEA-002401-PIP01-18](#)

---

Treatment of all conditions included in the category of malignant neoplasms (except central nervous system tumours, haematopoietic and lymphoid tissue neoplasms) overexpressing FGFR2b

Day 30 discussion

**Action:** For discussion

Oncology

### 3.1.36. [Autologous dendritic cells pulsed with allogeneic tumour cell lysate - Orphan - EMEA-002381-PIP01-18](#)

---

Amphera BV; Treatment of malignant mesothelioma

Day 30 discussion

**Action:** For discussion

Oncology

3.1.37. Autologous T lymphocyte-enriched population of cells transduced with a lentiviral vector encoding a chimeric antigen receptor targeting human B cell maturation antigen with 4-1BB and CD3-zeta intracellular signaling domains - Orphan - EMEA-002369-PIP01-18

---

Celgene Europe Limited; Treatment of mature B-cell neoplasms / Treatment of pediatric BCMA+ relapsed or refractory B non-Hodgkin lymphoma

Day 30 discussion

**Action:** For discussion

Oncology

3.1.38. Binimetinib - EMEA-001454-PIP05-18

---

Treatment of colorectal carcinoma

Day 30 discussion

**Action:** For discussion

Oncology

3.1.39. Brentuximab vedotin - Orphan - EMEA-000980-PIP04-18

---

Takeda Pharma A/S; Treatment of Mature T and NK neoplasms

Day 30 discussion

**Action:** For discussion

Oncology

3.1.40. Encorafenib - EMEA-001588-PIP03-18

---

Treatment of colorectal carcinoma

Day 30 discussion

**Action:** For discussion

Oncology

3.1.41. Pamiparib - EMEA-002389-PIP01-18

---

Gastric Neoplasms Malignant

Day 30 discussion

**Action:** For discussion

Oncology

### 3.1.42. Selinexor - Orphan - EMEA-002387-PIP01-18

---

Karyopharm Europe GmbH; Relapse/ Refractory Multiple myeloma

Day 30 discussion

**Action:** For discussion

Oncology

### 3.1.43. Diphtheria Toxin Interleukin-3 Fusion Protein; Orphan - EMEA-002244-PIP02-18

---

Stemline Therapeutics, Inc.; Treatment of blastic plasmacytoid dendritic cell neoplasm.

Day 30 discussion

**Action:** For discussion

Oncology

### 3.1.44. Telisotuzumab vedotin - EMEA-002361-PIP01-18

---

Lung carcinoma (small cell and non-small cell carcinoma)

Day 30 discussion

**Action:** For discussion

Oncology

### 3.1.45. Vinorelbine Tartrate (liposomal) - EMEA-002365-PIP01-18

---

Treatment of osteosarcomas, treatment of rhabdomyosarcoma, treatment of Ewing's sarcoma, treatment of non-rhabdomyosarcoma soft-tissue sarcomas / treatment of relapsed or refractory Ewing's sarcoma, treatment of relapsed or refractory rhabdomyosarcoma, treatment of relapsed or refractory osteosarcomas, treatment of relapsed or refractory non-rhabdomyosarcoma soft-tissue sarcomas

Day 30 discussion

**Action:** For discussion

Oncology

### 3.1.46. Eflapegrastim - EMEA-002385-PIP01-18

---

Treatment of Chemotherapy- Induced Neutropenia / Decrease the incidence of infection, as manifested by febrile neutropenia, in patients with non-myeloid malignancies receiving myelosuppressive anti-cancer drugs associated with a clinically significant incidence of febrile neutropenia.

Day 30 discussion

**Action:** For discussion

### 3.1.47. [Benzimidazole-containing ENaC inhibitor - EMEA-002394-PIP01-18](#)

---

Treatment of cystic fibrosis / indicated to improve lung function and reduce pulmonary exacerbations for patients with CF in conjunction with standard therapies.

Day 30 discussion

**Action:** For discussion

Pneumology - Allergology

### 3.1.48. [EMEA-002398-PIP01-18](#)

---

Treatment of cystic fibrosis in individuals with cystic fibrosis who are homozygous for the F508del mutation and are receiving treatment with a CFTR modulator

Day 30 discussion

**Action:** For discussion

Pneumology - Allergology

### 3.1.49. [Nintedanib - Orphan - EMEA-001006-PIP05-18](#)

---

Boehringer Ingelheim International GmbH; Treatment of fibrosing Interstitial Lung Diseases (ILD) / Treatment of fibrosing Interstitial Lung Diseases (ILD) in paediatric patients

Day 30 discussion

**Action:** For discussion

Pneumology - Allergology / Oncology

### 3.1.50. [EMEA-002373-PIP01-18](#)

---

Schizophrenia

Day 30 discussion

**Action:** For discussion

Psychiatry

## 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. Lamivudine (3TC) / Dolutegravir (DTG) - EMEA-C1-001940-PIP01-16-M01

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

Day 30 discussion

**Action:** For discussion

Infectious Diseases

### 3.2.2. Amikacin (sulfate) / Amikacin - EMEA-C3-000525-PIP01-08-M04

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Insmed Limited; Treatment of Pseudomonas aeruginosa lung infection / colonisation in cystic fibrosis patients

Day 30 discussion

**Action:** For discussion

Infectious Diseases / Pneumology - Allergology

### 3.2.3. Galcanezumab - EMEA-C4-001860-PIP03-16-M01

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Eli Lilly and Company Limited; Prevention of migraine headaches

Day 30 discussion

**Action:** For discussion

Neurology

### 3.2.4. Lacosamide - EMEA-C-000402-PIP02-11-M05

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

Day 30 discussion

**Action:** For discussion

Neurology

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

### 3.3.1. Omadacycline - EMEA-000560-PIP03-15-M01

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Paratek UK Limited; Treatment of bacterial pneumonia, Treatment of acute bacterial skin and skin structure infections (ABSSSI)

Day 30 discussion

**Action:** For discussion

Infectious Diseases

## 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 18 September 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

#### 6.1.1. Trifluridine and Tipiracil Hydrochloride – EMEA-13-2018

Les Laboratoires Servier; The class of pyrimidine- and pyrimidine analogue-containing medicinal products for treatment of intestinal malignant neoplasms/ Treatment of adult patients with metastatic gastric cancer who have received at least two prior regimens

**Action:** For adoption

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

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

#### 7.1.1. Rivaroxaban - EMEA-000430-PIP01-08-M10<sup>1</sup>

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Bayer AG; Cardiovascular Diseases

Proposed indication: prevention of VTE risk in ambulatory cancer patients initiating systemic cancer therapy and at high risk for VTE

**Action:** For adoption

## 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

### 9.2. 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

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

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CHMP/PDCO joint session

**Action:** For discussion

#### 9.2.3. Concept paper on the need for revision of the guideline on the investigation of medicinal products in the term and preterm neonate

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**Action:** For adoption

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<sup>1</sup> Correction to the name of the active substance

### **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

**Action:** For information

### **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**

#### **9.5.1. WHO-convened PAWG - Research Toolkit for Paediatric Antiretroviral Drug and Formulation Development – response to the WHO PAWG**

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PDCO member: Dirk Mentzer;

**Action:** For information

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

None

### **9.7. PDCO work plan**

None

### **9.8. Planning and reporting**

None

## 10. Any other business

### 10.1.1. Update on relocation to Amsterdam

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**Action:** For information

### 10.1.2. Training on paediatric requirements/legislations/initiatives in other regions – US

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**Action:** For information

### 10.1.3. Paediatric medicines Regulators' Network (PmRN) - WHO interactions

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**Action:** For information

### 10.1.4. Priority needs and plans for training 2018 – 2020

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**Action:** For information

### 10.1.5. EC/EMA action plan to further improve the implementation of the Paediatric Regulation

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Scope: Outcomes and action plan

**Action:** for adoption

### 10.1.6. Report from the SRLM Oslo meeting

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**Action:** for information

## 11. Breakout sessions

### 11.1.1. Paediatric oncology

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

### 11.1.2. Neonatology

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

### 11.1.3. Inventory

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

## 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/)