



EUROPEAN MEDICINES AGENCY  
SCIENCE MEDICINES HEALTH

28 May 2018  
EMA/357098/2018  
Inspections, Human Medicines Pharmacovigilance and Committees Division

## Paediatric Committee (PDCO)

Draft agenda for the meeting on 29 May-01 June 2018

Chair: Dirk Mentzer – Vice-Chair: Koenraad Norga

29 May 2018, 14:00- 19:00, room 03-A

30 May 2018, 08:30- 19:00, room 03-A

31 May 2018, 08:30- 19:00, room 03-A

01 June 2018, 08:30 - 13:00, room 03-A

### 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 29 May - 01 June 2018. See May 2018 PDCO minutes (to be published post June 2018 PDCO meeting).

### 1.2. Adoption of agenda

PDCO agenda for 29 May – 01 June 2018

### 1.3. Adoption of the minutes

PDCO minutes for 24-27 April 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. [Bimekizumab - EMEA-002189-PIP01-17](#)

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

Day 120 opinion

**Action:** For adoption

Dermatology

#### 2.1.2. [EMEA-002216-PIP01-17](#)

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Treatment of Atopic Dermatitis

Day 120 opinion

**Action:** For adoption

Dermatology



### 2.1.3. Dasiglucagon - Orphan - EMEA-002233-PIP01-17

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Zealand Pharma A/S; Treatment of hypoglycaemia

Day 120 opinion

**Action:** For adoption

Endocrinology-Gynaecology-Fertility-Metabolism

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

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Treatment of Ulcerative Colitis

Day 120 opinion

**Action:** For adoption

Gastroenterology-Hepatology

### 2.1.5. Itacitinib - Orphan - EMEA-002178-PIP01-17

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

Day 120 opinion

**Action:** For adoption

Immunology-Rheumatology-Transplantation

### 2.1.6. Recombinant IgG degrading enzyme of Streptococcus pyogenes - Orphan - EMEA-002183-PIP01-17

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Hansa Medical AB; Patients with chronic kidney disease in need of kidney transplantation / Prevention of graft rejection following solid organ transplantation

Day 120 opinion

**Action:** For adoption

Immunology-Rheumatology-Transplantation

### 2.1.7. The whole range of unmanipulated autologous mononuclear cells derived from human umbilical cord blood - Orphan - EMEA-001799-PIP02-17

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

Day 120 opinion

**Action:** For adoption

Neonatology - Paediatric Intensive Care

#### 2.1.8. EMEA-002184-PIP01-17

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Treatment of obstructive sleep apnoea, Treatment of narcolepsy / Treatment of excessive daytime sleepiness in narcolepsy patients

Day 120 opinion

**Action:** For adoption

Neurology

#### 2.1.9. Palbociclib - EMEA-002146-PIP01-17

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Treatment of Ewing sarcoma / treatment of refractory or recurrent Ewing sarcoma

Day 120 opinion

**Action:** For adoption

Oncology

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

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Prevention of rabies disease, treatment of exposure to rabies virus

Day 120 opinion

**Action:** For adoption

Vaccines

#### 2.1.11. Allogeneic bone-marrow derived adherent, ex-vivo expanded multipotent adult progenitor cells product - EMEA-002317-PIP01-17

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Acute ischaemic stroke

Day 60 opinion

**Action:** For adoption

Cardiovascular Diseases

#### 2.1.12. Dapagliflozin - EMEA-000694-PIP03-17

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I50 Heart Failure

Day 60 opinion

**Action:** For adoption

Cardiovascular Diseases

#### 2.1.13. Moxonidine - EMEA-002275-PIP01-17

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Treatment of Hypertension

Day 60 opinion

**Action:** For adoption

Cardiovascular Diseases

#### 2.1.14. Trandolapril - EMEA-002274-PIP01-17

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Mild or moderate hypertension

Day 60 opinion

**Action:** For adoption

Cardiovascular Diseases

#### 2.1.15. Patidegib - EMEA-002322-PIP01-17

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Treatment of basal cell carcinoma (BCC)

Day 60 opinion

**Action:** For adoption

Dermatology / Oncology

#### 2.1.16. Bilastine - EMEA-000347-PIP03-18

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Treatment of allergic rhinoconjunctivitis /Treatment of urticaria

Day 60 opinion

**Action:** For adoption

Dermatology / Pneumology - Allergology / Oto-rhino-laryngology

#### 2.1.17. Abatacept - EMEA-000118-PIP04-17

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Treatment of childhood-onset of Sjögren's Syndrome

Day 60 opinion

**Action:** For adoption

Immunology-Rheumatology-Transplantation

#### 2.1.18. Liposomal ciclosporin A (L-CsA) - Orphan - EMEA-002344-PIP01-18

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Breath Therapeutics GmbH; Treatment of Bronchiolitis obliterans Syndrome (BOS)

Day 60 opinion

**Action:** For adoption

Immunology-Rheumatology-Transplantation

#### 2.1.19. Upadacitinib Hemihydrate - EMEA-001741-PIP05-17

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Treatment of Vasculitides

Day 60 opinion

**Action:** For adoption

Immunology-Rheumatology-Transplantation

#### 2.1.20. Reltecimod - Orphan - EMEA-002325-PIP01-18

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Atox Bio Ltd.; Treatment for necrotizing soft tissue infections

Day 60 opinion

**Action:** For adoption

Infectious Diseases

#### 2.1.21. Fostamatinib - EMEA-001196-PIP02-17

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Treatment of immune thrombocytopenia (idiopathic thrombocytopenic purpura)

Day 60 opinion

**Action:** For adoption

Other

#### 2.1.22. Nitrous oxide - EMEA-002340-PIP01-18

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Anesthesia, analgesia / sedation / In analgesia / sedation in all conditions in which pain relief / sedation with rapid onset and rapid fall in effect is required (short-term surgical interventions, traumatology, burns, dentistry, otorhinolaryngology, childbirth) / Under anesthesia, in combination with other anesthetics administered by inhalation or intravenously.

Day 60 opinion

**Action:** For adoption

Pain / Anaesthesiology

## 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. Osilodrostat - EMEA-C1-000315-PIP02-15-M01

---

Novartis Europharm Limited; Treatment of adrenal cortical hyperfunctions

Day 60 letter

**Action:** For adoption

Endocrinology-Gynaecology-Fertility-Metabolism

### 2.2.2. Birch pollen extract (Betula verrucosa) - EMEA-C1-001879-PIP01-15-M01

---

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

Day 60 letter

**Action:** For adoption

Pneumology - Allergology

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

### 2.3.1. Bempedoic acid - EMEA-001872-PIP01-15-M01

---

Esperion Therapeutics, Inc.; Treatment of elevated cholesterol / Treatment of heterozygous and homozygous familial hypercholesterolemia

Day 60 opinion

**Action:** For adoption

Cardiovascular Diseases

### 2.3.2. Regadenoson - EMEA-000410-PIP01-08-M03

---

GE Healthcare AS; Myocardial perfusion disturbances / Diagnostic evaluation of myocardial perfusion disturbances

Day 60 opinion

**Action:** For adoption

Cardiovascular Diseases

### 2.3.3. Ticagrelor - EMEA-000480-PIP01-08-M11

---

AstraZeneca AB; thromboembolic events (children), acute coronary syndrome, history of

myocardial infarction / reduction in occurrence of vaso-occlusive crises in paediatric patients with sickle cell disease

Day 60 opinion

**Action:** For adoption

Cardiovascular Diseases / Haematology-Hemostaseology

#### 2.3.4. Brodalumab - EMEA-001089-PIP02-13-M01

LEO Pharma A/S; Treatment of psoriasis

Day 60 opinion

**Action:** For adoption

Dermatology

#### 2.3.5. Ligelizumab - EMEA-001811-PIP02-15-M02

Novartis Europharm Ltd.; Treatment of chronic spontaneous urticaria

Day 60 opinion

**Action:** For adoption

Dermatology

#### 2.3.6. 2-hydroxypropyl- $\beta$ -cyclodextrin (HP- $\beta$ -CD) - Orphan - EMEA-001866-PIP01-15-M02

Mallinckrodt Pharmaceuticals Ireland Ltd; Treatment of Niemann-Pick disease, type C / Treatment of progressive neurological manifestations in children and adolescent patients with Niemann-Pick disease, type C

Day 60 opinion

**Action:** For adoption

Endocrinology-Gynaecology-Fertility-Metabolism

#### 2.3.7. Empagliflozin - EMEA-000828-PIP01-09-M07

Boehringer Ingelheim International GmbH; Treatment of type 2 diabetes mellitus

Day 60 opinion

**Action:** For adoption

Endocrinology-Gynaecology-Fertility-Metabolism

#### 2.3.8. Glycerol phenylbutyrate - Orphan - EMEA-000297-PIP02-12-M02

Horizon Pharma Ireland Limited; E72.2 Urea cycle disorders / indicated for use as

adjunctive therapy for chronic management of patients with urea cycle disorders (UCDs) including deficiencies of carbamoyl phosphate-synthase-I (CPS), ornithine carbamoyltransferase (OTC), argininosuccinate synthetase (ASS), argininosuccinate lyase (ASL), arginase I (ARG) and ornithine translocase deficiency hyperornithinaemia-hyperammonaemia homocitrullinuria syndrome (HHH) who cannot be managed by dietary protein restriction and/or amino acid supplementation alone.

Day 60 opinion

**Action:** For adoption

Endocrinology-Gynaecology-Fertility-Metabolism

### 2.3.9. Linagliptin - EMEA-000498-PIP01-08-M08

Boehringer Ingelheim International GmbH; Treatment of type 2 diabetes mellitus

Day 60 opinion

**Action:** For adoption

Endocrinology-Gynaecology-Fertility-Metabolism

### 2.3.10. EMEA-001356-PIP02-12-M02

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

Day 60 opinion

**Action:** For adoption

Gastroenterology-Hepatology

### 2.3.11. Vonicog alfa - Orphan - EMEA-001164-PIP01-11-M02

Baxalta Innovations GmbH; Von Willebrand Disease / Treatment and control of haemorrhage (spontaneous and surgical) and prevention of bleeding in surgery in paediatric patients (age of < 18 years) diagnosed with VWD when desmopressin (DDAVP) treatment alone is ineffective or not indicated.

Day 60 opinion

**Action:** For adoption

Haematology-Hemostaseology

### 2.3.12. Eculizumab - Orphan - EMEA-000876-PIP05-15-M03

Alexion Europe SAS; Myasthenia Gravis / Treatment of Refractory Generalized yasthenia Gravis

Day 60 opinion

**Action:** For adoption

Immunology-Rheumatology-Transplantation

2.3.13. [Expanded donor-derived allogenic T cells transduced with the retroviral vector expressing the transgenes for inducible caspase9 and the truncated CD19 selectable marker - Orphan - EMEA-001869-PIP01-15-M01](#)

---

Bellicum Pharma Ltd; Adjunctive treatment in haematopoietic stem cell transplantation

Day 60 opinion

**Action:** For adoption

Immunology-Rheumatology-Transplantation

2.3.14. [Rimiducid - Orphan - EMEA-001870-PIP01-15-M01](#)

---

Bellicum Pharma Ltd.; Treatment of graft versus host disease

Day 60 opinion

**Action:** For adoption

Immunology-Rheumatology-Transplantation

2.3.15. [Tofacitinib - EMEA-000576-PIP01-09-M09](#)

---

Pfizer Limited; 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

2.3.16. [Arimoclomol citrate - Orphan - EMEA-001748-PIP01-15-M01](#)

---

Orphazyme A/S; Treatment of Niemann-Pick Disease, Type C

Day 60 opinion

**Action:** For adoption

Neurology

2.3.17. [Domagrozumab - Orphan - EMEA-001763-PIP01-15-M02](#)

---

Pfizer Ltd; Duchenne Muscular Dystrophy

Day 60 opinion

**Action:** For adoption

Neurology



### 2.3.18. Pitolisant - Orphan - EMEA-001176-PIP01-11-M03

---

BIOPROJET PHARMA; Narcolepsy / Narcolepsy with or without cataplexy

Day 60 opinion

**Action:** For adoption

Neurology

### 2.3.19. Tasimelteon - Orphan - EMEA-001531-PIP01-13-M04

---

Vanda Pharmaceuticals; ICD-10 G47.24 Circadian rhythm sleep disorder, free-running type (Non-24) / Non24-Hour Sleep-Wake Disorder (Non-24) in the totally blind

Day 60 opinion

**Action:** For adoption

Neurology

### 2.3.20. Cobimetinib - EMEA-001425-PIP01-13-M03

---

Roche Registration Limited; 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 60 opinion

**Action:** For adoption

Oncology

### 2.3.21. Larotrectinib - Orphan - EMEA-001971-PIP02-16-M01

---

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 60 opinion

**Action:** For adoption

Oncology

### 2.3.22. Sirolimus - Orphan - EMEA-001416-PIP01-12-M02

---

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

Day 60 opinion

**Action:** For adoption

Ophthalmology

2.3.23. Autologous CD34+ enriched cell fraction that contains CD34+ cells transduced with lentiviral vector that encodes for the human ARSA cDNA sequence - Orphan - EMEA-001765-PIP02-15-M02

---

GlaxoSmithKline Trading Services Limited; Treatment of Metachromatic leukodystrophy (MLD)

Day 60 opinion

**Action:** For adoption

Other

2.3.24. Calcium chloride / Aprotinin / Fibrinogen / Thrombin - EMEA-001079-PIP01-10-M04

---

Kedrion S.p.A.; T81.0 Haemorrhage and haematoma complicating a procedure, not elsewhere classified / Treatment and prevention of haemorrhage resulting from a surgical procedure

Day 60 opinion

**Action:** For adoption

Other

2.3.25. Mexiletine hydrochloride - Orphan - EMEA-002012-PIP01-16-M01

---

Lupin (Europe) Ltd.; Treatment of myotonic disorders / Symptomatic treatment of myotonic disorders

Day 60 opinion

**Action:** For adoption

Other

2.3.26. Gabapentin - EMEA-001310-PIP01-12-M03

---

PHARM Srl; Treatment of chronic pain / Treatment of chronic pain in paediatric patients aged from 3 months to less than 18 years

Day 60 opinion

**Action:** For adoption

Pain

### 2.3.27. Tezepelumab - EMEA-001613-PIP01-14-M01

---

AstraZeneca AB; Treatment of asthma / Tezepelumab is indicated as add-on maintenance treatment of patients with severe asthma aged 5 years and older.

Day 60 opinion

**Action:** For adoption

Pneumology - Allergology

### 2.3.28. Potassium hydrogen carbonate / Potassium citrate monohydrate - EMEA-001535-PIP01-13-M01

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Advicenne; Cystinuria (ICD 10: E72.0)

Day 60 opinion

**Action:** For adoption

Uro-nephrology

### 2.3.29. Sucroferric oxyhydroxide - EMEA-001061-PIP01-10-M03

---

Vifor Fresenius Medical Care Renal Pharma France; Hyperphosphataemia / Control of serum phosphorus levels in paediatric and adolescent subjects with chronic kidney disease (CKD)

Day 60 opinion

**Action:** For adoption

Uro-nephrology

### 2.3.30. 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 based on annual recommendations by WHO, CHMP (EU) and other regional or local authorities - EMEA-001782-PIP01-15-M03

---

Abbott Biologicals B.V.; Prevention of Influenza infection / Prophylaxis of influenza; especially in those who run an increased risk of associated complications

Day 60 opinion

**Action:** For adoption

Vaccines

### 2.3.31. Recombinant Varicella Zoster Virus (VZV) glycoprotein E antigen - EMEA-001426-PIP01-13-M02

---

GlaxoSmithKline Biologicals SA; Prevention of VZV reactivation / Prevention of herpes zoster in immunocompromised subjects aged 1 to 17 years

Day 60 opinion

**Action:** For adoption

Vaccines

#### 2.3.32. Tocilizumab - EMEA-000309-PIP04-17-M01

---

Roche Registration Limited; Treatment of chimeric antigen receptor (CAR) T cell-induced severe or life-threatening cytokine release syndrome (CRS) in adults and paediatric patients 2 years of age and older

Day 30 opinion

**Action:** For adoption

Immunology-Rheumatology-Transplantation

### 2.4. Opinions on Re-examinations

None

### 2.5. Opinions on Review of Granted Waivers

None

### 2.6. Finalisation and adoption of opinions

None

### 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. Crisaborole- EMEA-C3-002065-PIP01-16-M01

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Pfizer Ltd.; Treatment of atopic dermatitis

Day 1 letter

**Action:** For information

Dermatology

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

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Wilson Therapeutics AB; Treatment of Wilson disease

Day 90 discussion

**Action:** For discussion

Endocrinology-Gynaecology-Fertility-Metabolism

#### 3.1.2. His-Ser-Gln-Gly-Thr-Phe-Thr-Ser-Asp-Lys( $\gamma$ -Glu-palmitoyl)-Ser-Glu-Tyr-Leu-Asp-Ser- Glu-Arg-Ala-Arg-Asp-Phe-Val-Ala-Trp-Leu-Glu-Ala-Gly-Gly-OH - EMEA-002287-PIP01-17

---

Treatment of Type 2 Diabetes Mellitus

Day 90 discussion

**Action:** For discussion

Endocrinology-Gynaecology-Fertility-Metabolism

#### 3.1.3. Alicaforsen - Orphan - EMEA-002060-PIP02-17

---

Atlantic Pharmaceuticals (Holdings) Ltd; Treatment of gastrointestinal procedural complications / Treatment of active episodes of antibiotic refractory pouchitis

Day 90 discussion

Gastroenterology-Hepatology

#### 3.1.4. Risankizumab - EMEA-001776-PIP03-17

---

Crohn's Disease

Day 90 discussion

**Action:** For discussion

Gastroenterology-Hepatology

#### 3.1.5. Risankizumab - EMEA-001776-PIP04-17

---

Ulcerative Colitis

Day 90 discussion

**Action:** For discussion

Gastroenterology-Hepatology

### 3.1.6. Cefiderocol - EMEA-002133-PIP01-17

---

Treatment of Gram-negative bacterial infections

Day 90 discussion

**Action:** For discussion

Infectious Diseases

### 3.1.7. Glasdegib maleate - Orphan - EMEA-002199-PIP01-17

---

Pfizer Limited; Treatment of acute myeloid leukaemia (AML)

Day 90 discussion

**Action:** For discussion

Oncology

### 3.1.8. Olaparib - Orphan - EMEA-002269-PIP01-17

---

AstraZeneca AB; Treatment of all conditions included in the category of malignant neoplasms (except central nervous system [CNS], haematopoietic, and lymphoid tissue). / Treatment of paediatric patients from 6 months to ≤18 years old with homologous recombination repair (HRR) mutated solid tumours

Day 90 discussion

**Action:** For discussion

Oncology

### 3.1.9. Recombinant humanised monoclonal IgG2 lambda antibody against human sclerostin - Orphan - EMEA-002169-PIP01-17

---

Mereo Biopharma 3 Ltd; Treatment of osteogenesis imperfecta / Treatment of osteogenesis imperfecta, types 1, 3 and 4

Day 90 discussion

**Action:** For discussion

Other

### 3.1.10. Ferric Pyrophosphate Citrate - EMEA-002261-PIP01-17

---

Iron deficient anaemia / Treatment of iron deficient anaemia in haemodialysis patients

Day 90 discussion

**Action:** For discussion

Uro-nephrology / Haematology-Hemostaseology

### 3.1.11. 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 90 discussion

**Action:** For discussion

Vaccines / Infectious Diseases

### 3.1.12. 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 90 discussion

**Action:** For discussion

Vaccines / Infectious Diseases

### 3.1.13. Mosaic gp140Clade C 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 90 discussion

**Action:** For discussion

Vaccines / Infectious Diseases

### 3.1.14. Evinacumab - EMEA-002298-PIP01-17

---

Treatment of elevated cholesterol / Treatment of homozygous familial hypercholesterolemia (HoFH)

Day 60 discussion

**Action:** For discussion

Endocrinology-Gynaecology-Fertility-Metabolism

### 3.1.15. Ianalumab - EMEA-002338-PIP01-18

---

Autoimmune hepatitis (AIH) / Treatment of autoimmune hepatitis in patients aged 12 years to <18 years in whom steroids and/or azathioprine are contraindicated, are not tolerated, or do not provide an adequate response

Day 60 discussion

**Action:** For discussion

Gastroenterology-Hepatology

### 3.1.16. EMEA-002240-PIP02-17

---

Treatment of Urinary Tract Infections

Day 60 discussion

**Action:** For discussion

Infectious Diseases

### 3.1.17. EMEA-001970-PIP02-17

---

Treatment of Clostridium difficile infection / indicated to reduce recurrence of Clostridium difficile infection (CDI) in paediatric patients who have received antibacterial drug treatment for recurrent CDI.

Day 60 discussion

**Action:** For discussion

Infectious Diseases

### 3.1.18. Isoflurane - EMEA-002320-PIP01-17

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Sedation

Day 60 discussion

**Action:** For discussion

Neonatology - Paediatric Intensive Care

### 3.1.19. Bupivacaine - EMEA-000877-PIP03-17

---

Postsurgical analgesia

Day 60 discussion

**Action:** For discussion

Pain

### 3.1.20. Calcifediol - EMEA-002093-PIP02-17

---

Secondary hyperparathyroidism (SHPT) / treatment of secondary hyperparathyroidism (SHPT) in non-dialysis chronic kidney disease (ND-CKD) patients with low serum 25-hydroxyvitamin D levels

Day 60 discussion

**Action:** For discussion

Uro-nephrology



### 3.1.21. [Amlodipine / irbesartan - EMEA-002352-PIP01-18](#)

---

Treatment of essential hypertension

Day 30 discussion

**Action:** For discussion

Cardiovascular Diseases

### 3.1.22. [EMEA-002329-PIP01-18](#)

---

Treatment of chronic hand eczema

Day 30 discussion

**Action:** For discussion

Dermatology

### 3.1.23. [Givosiran sodium - Orphan - EMEA-002048-PIP02-18](#)

---

Alnylam UK Limited; Treatment of Acute Hepatic Porphyria (AHP)

Day 30 discussion

**Action:** For discussion

Endocrinology-Gynaecology-Fertility-Metabolism

### 3.1.24. [Venglustat - Orphan - EMEA-001716-PIP03-18](#)

---

Genzyme Europe B.V.; ICD-10: Q61.2; Polycystic kidney, autosomal dominant; Congenital malformations of the urinary system (Q60-Q64); Polycystic kidney, adult type.

Day 30 discussion

**Action:** For discussion

Endocrinology-Gynaecology-Fertility-Metabolism / Uro-nephrology

### 3.1.25. [EMEA-002362-PIP01-18](#)

---

Hereditary factor IX deficiency (Haemophilia B) / Prophylaxis of haemophilia B (hereditary factor IX deficiency)

Day 30 discussion

**Action:** For discussion

Haematology-Hemostaseology

### 3.1.26. Luspatercept - EMEA-001521-PIP02-18

---

Treatment of Myelofibrosis

Day 30 discussion

**Action:** For discussion

Haematology-Hemostaseology

### 3.1.27. Voxelotor - Orphan - EMEA-002356-PIP01-18

---

SynteractHCR Deutschland GmbH; Treatment of sickle cell disease

Day 30 discussion

**Action:** For discussion

Haematology-Hemostaseology

### 3.1.28. Ustekinumab - EMEA-000311-PIP06-18

---

Treatment of systemic lupus erythematosus (SLE)

Day 30 discussion

**Action:** For discussion

Immunology-Rheumatology-Transplantation

### 3.1.29. Nacubactam - EMEA-002339-PIP01-18

---

Treatment of Gram-negative bacterial infections. / Nacubactam co-administered with meropenem is indicated for the treatment of serious infections including cUTI, HAP, VAP, and BSI caused by Gram-negative bacteria in patients with limited treatment options.

Day 30 discussion

**Action:** For discussion

Infectious Diseases

### 3.1.30. Abemaciclib - EMEA-002342-PIP01-18

---

Ewing's Sarcoma

Day 30 discussion

**Action:** For discussion

Oncology

### 3.1.31. EMEA-002348-PIP01-18

---

B-cell Acute Lymphoblastic Leukemia / Treatment of relapse or refractory B-cell acute

lymphoblastic leukemia

Day 30 discussion

**Action:** For discussion

Oncology

---

3.1.32. [Autologous T cells transduced with retroviral vector encoding an anti-CD19 CD28/CD3- zeta chimeric antigen receptor - Orphan - EMEA-002335-PIP01-18](#)

---

Kite Pharma EU B.V.; Treatment of Mantle Cell Lymphoma

Day 30 discussion

**Action:** For discussion

Oncology

---

3.1.33. [Iodine \(131-I\) murine IgG1 monoclonal antibody against B7-H3 - Orphan - EMEA-002101-PIP02-18](#)

---

Y-mAbs Therapeutics A/S; Treatment of pediatric neuroblastoma patients with CNS relapse as evidenced by CNS/LM metastases

Day 30 discussion

**Action:** For discussion

Oncology

---

3.1.34. [Navitoclax - EMEA-000478-PIP02-18](#)

---

Treatment of myelofibrosis

Day 30 discussion

**Action:** For discussion

Oncology

---

3.1.35. [Pegvorhialuronidase alfa - Orphan - EMEA-001883-PIP03-17](#)

---

Halozyme Inc; Treatment of all conditions included in the category of malignant neoplasms (except central nervous system tumours, haematopoietic and lymphoid tissue neoplasms). / Pegvorhialuronidase alfa is indicated in combination with cytotoxic cancer therapies for the treatment of paediatric patients aged 6 months to less than 18 years with relapsed or refractory solid tumours that accumulate high levels of hyaluronan.

Day 30 discussion

**Action:** For discussion

Oncology

### 3.1.36. Veliparib - Orphan - EMEA-000499-PIP05-18

---

AbbVie Ltd; Treatment of ovarian carcinoma, Treatment of fallopian tube carcinoma, Treatment of peritoneal carcinoma

Day 30 discussion

**Action:** For discussion

Oncology

### 3.1.37. Anti-alpha v beta 6 monoclonal antibody - Orphan - EMEA-002332-PIP01-18

---

Biogen Idec Ltd; Idiopathic Pulmonary Fibrosis

Day 30 discussion

Action: For discussion

Pneumology - Allergology

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

---

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

### 3.1.39. EMEA-002121-PIP02-18

---

Treatment of insomnia

Day 30 discussion

**Action:** For discussion

Psychiatry

### 3.1.40. Rapastinel - EMEA-002357-PIP01-18

---

Major depressive disorder

Day 30 discussion

**Action:** For discussion

Psychiatry

### 3.1.41. 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

---

Prevention of influenza infection

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. Testosterone - EMEA-C2-001529-PIP02-14-M01

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Acerus Biopharma Inc.; Treatment of male hypogonadism

Day 30 discussion

**Action:** For discussion

Endocrinology-Gynaecology-Fertility-Metabolism

### 3.2.2. Nonacog gamma - EMEA-C-001139-PIP01-11-M02

---

Baxalta Innovations GmbH; Treatment of haemophilia B (congenital factor IX deficiency)

Day 30 discussion

**Action:** For discussion

Haematology-Hemostaseology

### 3.2.3. Cilastatin sodium / relebactam / imipenem monohydrate - EMEA-C1-001809-PIP01-15

---

Merck Sharp & Dohme (Europe), Inc.; Treatment of Gram-negative bacterial infections

Day 30 discussion

**Action:** For discussion

Infectious Diseases

### 3.2.4. Everolimus - EMEA-C-000019-PIP08-12-M03

---

Novartis Europharm Limited; Treatment of Tuberous Sclerosis Complex

Day 30 discussion

**Action:** For discussion

Neurology

3.2.5. [Autologous CD4+ and CD8+ cells expressing a CD19-specific chimeric antigen receptor - EMEA-C1-001995-PIP01-16-M01](#)

---

Celgene Europe Limited; Treatment of B-lymphoblastic leukemia/lymphoma

Day 30 discussion

**Action:** For discussion

Oncology

3.2.6. [Quizartinib - EMEA-C1-001821-PIP01-15-M01](#)

---

Daiichi Sankyo Europe GmbH; 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. [Landiolol hydrochloride - EMEA-001150-PIP02-13-M02](#)

---

AOP Orphan Pharmaceuticals AG; Treatment of supraventricular arrhythmias

Day 30 discussion

**Action:** For discussion

Cardiovascular Diseases

3.3.2. [Tralokinumab - EMEA-001900-PIP02-17-M01](#)

---

LEO Pharma A/S; Treatment of Atopic Dermatitis

Day 30 discussion

**Action:** For discussion

Dermatology

### 3.3.3. Exenatide - EMEA-001755-PIP01-15-M01

---

LES LABORATOIRES SERVIER; treatment of type 2 diabetes mellitus / Treatment of type 2 diabetes mellitus in patients from 10 to less than 18 years old

Day 30 discussion

**Action:** For discussion

Endocrinology-Gynaecology-Fertility-Metabolism

### 3.3.4. Olipudase alfa - Orphan - EMEA-001600-PIP01-13-M01

---

Genzyme Europe B.V.; ICD-10: E75.2; Endocrine, nutritional and metabolic diseases, Metabolic disorders, Disorders of sphingolipid metabolism and other lipid storage disorders, Other sphingolipidosis, Niemann-Pick Disease.

Day 30 discussion

**Action:** For discussion

Endocrinology-Gynaecology-Fertility-Metabolism

### 3.3.5. Osilodrostat - Orphan - EMEA-000315-PIP02-15-M02

---

Novartis Europharm Limited; Treatment of adrenal cortical hyperfunctions / Treatment of Cushing's disease in adolescents and children aged 6 years and older

Day 30 discussion

**Action:** For discussion

Endocrinology-Gynaecology-Fertility-Metabolism

### 3.3.6. Romosozumab - EMEA-001075-PIP04-15-M01

---

UCB Pharma S.A.; Treatment of osteoporosis / Treatment of osteogenesis imperfecta, Treatment of glucocorticoid-induced osteoporosis

Day 30 discussion

**Action:** For discussion

Endocrinology-Gynaecology-Fertility-Metabolism

### 3.3.7. Roxadustat - EMEA-001557-PIP01-13-M02

---

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

Day 30 discussion

**Action:** For discussion

Haematology-Hemostaseology

### 3.3.8. Ciprofloxacin Hydrochloride - EMEA-001563-PIP02-15-M01

---

Aradigm Limited; Treatment of chronic pulmonary infections caused by Pseudomonas aeruginosa

Day 30 discussion

**Action:** For discussion

Infectious Diseases

### 3.3.9. Oseltamivir phosphate - EMEA-000365-PIP01-08-M10

---

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

### 3.3.10. Balovaptan - EMEA-001918-PIP01-15-M01

---

Roche Registration Ltd; ICD10 F84: Treatment of autism spectrum disorder / Treatment of core social and communication deficits in people with autism spectrum disorder aged 2 years or older

Day 30 discussion

**Action:** For discussion

Neurology

### 3.3.11. EMEA-002072-PIP01-16-M01

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Incyte Corporation; Treatment of select unresectable or metastatic solid tumours with epacadostat in combination with pembrolizumab in paediatric patients between the ages of 6 months and 18 years of age. / Select unresectable or metastatic solid tumours in paediatric patients >6 months and < 18 years

Day 30 discussion

**Action:** For discussion

Oncology

### 3.3.12. Avelumab (recombinant human monoclonal IgG1 antibody directed against Programmed Death Ligand-1 (anti-PD-L1); Orphan - EMEA-001849-PIP02-15-M02

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Merck KGaA; Treatment of all conditions included in the category of malignant neoplasms (except central nervous system tumors, haematopoietic and lymphoid tissue neoplasms), Treatment of malignant neoplasms of lymphoid tissue, Treatment of malignant



neoplasms of the central nervous system / Treatment of paediatric patients from birth to less than 18 years old with a refractory or relapsed tumour of the central nervous system or with a tumour of the central nervous system as part of first line treatment, Treatment of paediatric patients from birth to less than 18 years old with a relapsed or refractory solid tumour or with a solid tumour as part of the first line treatment, Treatment of paediatric patients from birth to less than 18 years old with a refractory or relapsed Hodgkin or non-Hodgkin lymphoma, or with Hodgkin or non-Hodgkin lymphoma as part of first line treatment

Day 30 discussion

**Action:** For discussion

Oncology

### 3.3.13. Eribulin - EMEA-001261-PIP01-11-M05

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Eisai Europe Ltd; Soft Tissue Sarcoma

Day 30 discussion

**Action:** For discussion

Oncology

### 3.3.14. Paclitaxel - EMEA-001308-PIP01-12-M02

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Celgene Europe Limited; Treatment of Solid malignant tumours / Treatment of a paediatric solid malignant tumour

Day 30 discussion

**Action:** For discussion

Oncology

### 3.3.15. Pixantrone (as dimaleate) - EMEA-000713-PIP02-10-M05

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CTI Life Sciences Limited; ICD-09. C83 Diffuse non-Hodgkin's Lymphoma (including C83.7 Burkitt Lymphoma, C83.5 Lymphoblastic Lymphoma, C83.3 Large-cell Lymphoma) / Treatment of Non-Hodgkin's Lymphoma

Day 30 discussion

**Action:** For discussion

Oncology

### 3.3.16. Andexanet alfa - EMEA-001902-PIP01-15-M03

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Portola Pharma UK Limited; prevention of factor Xa inhibitor associated haemorrhage, treatment of factor Xa inhibitor associated haemorrhage / For the reversal of anticoagulation due to direct and indirect factor Xa inhibitors in patients requiring urgent surgery, For the reversal of anticoagulation due to direct and indirect factor Xa inhibitors in patients experiencing an acute major bleeding episode

Day 30 discussion

**Action:** For discussion

Other

### 3.3.17. Febuxostat - EMEA-001417-PIP01-12-M04

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Menarini International Operations Luxembourg S.A.; Prevention/treatment of hyperuricemia / Prevention or treatment of hyperuricemia in patients at intermediate or high risk of Tumor Lysis Syndrome (TLS) affected by hematologic malignancies

Day 30 discussion

**Action:** For discussion

Other / Oncology

### 3.3.18. Benralizumab - EMEA-001214-PIP01-11-M08

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AstraZeneca AB; Treatment of asthma

Day 30 discussion

**Action:** For discussion

Pneumology - Allergology

### 3.3.19. Peanut Allergen Extract - EMEA-001481-PIP01-13-M02

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DBV Technologies S.A; Peanut allergy

Day 30 discussion

**Action:** For discussion

Pneumology - Allergology

### 3.3.20. Vortioxetine - EMEA-000455-PIP02-10-M04

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H. Lundbeck A/S; Major Depressive Disorder

Day 30 discussion

**Action:** For discussion

Psychiatry

### 3.3.21. Fc- and CDR-modified humanized monoclonal antibody against C5 - Orphan - EMEA-001943-PIP01-16-M01

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Alexion Europe SAS; Atypical Haemolytic Uremic Syndrome / Treatment of atypical Haemolytic Uremic Syndrome

Day 30 discussion

**Action:** For discussion

3.3.22. **Influenza virus surface antigens - A/turkey/Turkey/1/05 (H5N1) - EMEA-000599-PIP01-09-M06**

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Seqirus S.r.l.; Prevention of Influenza / Active immunisation against H5N1 subtype of Influenza A virus

Day 30 discussion

**Action:** For discussion

Vaccines

3.3.23. **Pandemic influenza vaccine (H5N1) (surface antigen, inactivated, adjuvanted) - EMEA-001830-PIP01-15-M01**

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Seqirus S.r.l.; Prevention of influenza / Prophylaxis of influenza in an officially declared pandemic situation

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 24 July 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

None

### 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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Joint CHMP/PDCO session

**Action:** For discussion

### **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**

None

### **9.5. Cooperation with International Regulators**

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

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PDCO member: Dirk Mentzer;

**Action:** For discussion

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

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Scope: Outcomes and action plan

**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/)