



EUROPEAN MEDICINES AGENCY  
SCIENCE MEDICINES HEALTH

3 November 2016  
EMA/PDCO/701773/2016 Rev.1  
Inspections, Human Medicines Pharmacovigilance and Committees Division

## Paediatric Committee (PDCO)

Draft agenda for the meeting on 8-11 November 2016

Chair: Dirk Mentzer

8 November 2016, 13:00 - 17:00, room 3F

9 November 2016, 08:30 - 19:00, room 3A

10 November 2016, 08:30 - 19:00, room 3A

11 November 2016, 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

### Welcome and declarations of interest of members, alternates and experts

Pre-meeting list of participants and restrictions in relation to declarations of interests applicable to the items of the agenda for the PDCO plenary session to be held 8-11 November 2016. See November 2016 PDCO minutes (to be published post December 2016 PDCO meeting).

### Adoption of agenda

PDCO Agenda for 8-11 November 2016.

### Adoption of the minutes

PDCO Minutes for 12-14 October 2016.

## 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. Antithrombin alfa - EMEA-001154-PIP02-15

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Treatment of congenital antithrombin deficiency, Treatment of acquired antithrombin deficiency (Preeclampsia), Treatment of acquired antithrombin deficiency (ECMO) / Prophylaxis of peri-partum thromboembolic events in congenital antithrombin deficient patients, Antithrombin supplementation during ECMO procedure, Treatment of pregnant women less than 30 weeks GA with preeclampsia to prolong gestation and decrease foetal and neonatal morbidity and mortality

Day 120 opinion

**Action:** For information; adopted via written procedure on 19 October 2016

Haematology-Hemostaseology

#### 2.1.2. Eculizumab - Orphan - EMEA-000876-PIP03-14

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Alexion Europe SAS; Neuromyelitis Optica Spectrum Disorders / Treatment of Relapsing Neuromyelitis Optica Spectrum Disorders in the paediatric population

Day 120 opinion

**Action:** For adoption

Immunology-Rheumatology-Transplantation

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### 2.1.3. Octenidine dihydrochloride - Orphan - EMEA-001384-PIP01-12

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Schülke & Mayr GmbH; Skin disinfection

Day 120 opinion

**Action:** For adoption

Neonatology - Paediatric Intensive Care

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### 2.1.4. synthetic surfactant protein B analogue / synthetic surfactant protein C analogue / 1-palmitoyl-2-oleoyl-sn-glycero-3-phosphoglycerol sodium salt / dipalmitoylphosphatidylcholine - Orphan - EMEA-001780-PIP01-15

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Chiesi Farmaceutici SpA; treatment of respiratory distress syndrome (RDS) / treatment of respiratory distress syndrome (RDS) in preterm neonates of less than 37 weeks of gestational age

Day 120 opinion

**Action:** For adoption

Neonatology - Paediatric Intensive Care

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### 2.1.5. Galcanezumab - EMEA-001860-PIP04-16

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Prophylactic treatment of cluster headache

Day 120 opinion

**Action:** For adoption

Neurology

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### 2.1.6. Dexamethasone / Povidone-Iodine - EMEA-001936-PIP01-16

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Treatment of Infectious conjunctivitis (adenoviral and bacterial)

Day 120 opinion

**Action:** For adoption

Ophthalmology

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### 2.1.7. lauromacrogol 400 - EMEA-002026-PIP02-16

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venous therapeutic procedures / sclerotherapy of varicose veins

Day 60 opinion



**Action:** For adoption

Cardiovascular Diseases

#### 2.1.8. Botulinum toxin, Type A - EMEA-002038-PIP01-16

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Treatment of glabellar lines

Day 60 opinion

**Action:** For adoption

Dermatology

#### 2.1.9. vemurafenib - EMEA-000978-PIP03-16

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Treatment of histiocytic disease

Day 60 opinion

**Action:** For adoption

Oncology

#### 2.1.10. Netarsudil - EMEA-002037-PIP01-16

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Open Angle Glaucoma / Ocular Hypertension

Day 60 opinion

**Action:** For adoption

Ophthalmology

#### 2.1.11. palonosetron / fosnetupitant (netupitant prodrug) - EMEA-001198-PIP02-16

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Chemotherapy-Induced Nausea and Vomiting

Day 60 opinion

**Action:** For adoption

Other

#### 2.1.12. Ibuprofen - EMEA-002017-PIP01-16

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Treatment of pain, Treatment of febrile disorders / Treatment of pain of non-serious arthritic conditions, Treatment of backache, Treatment of dental pain, Treatment of neuralgia, Treatment of headache, Treatment of rheumatic or muscular pain, Treatment of migraine, Treatment of dysmenorrhoea, Treatment of fever

Day 60 opinion

**Action:** For adoption

Other / Pain

2.1.13. [DNA plasmid encoding HPV type 18 consensus E6 and E7 proteins \(pGX3002\) / DNA plasmid encoding HPV type 16 consensus E6 and E7 proteins \(pGX3001\) - EMEA-002022-PIP01-16](#)

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Treatment of high grade squamous intraepithelial lesions (HSIL) of the cervix caused by HPV types 16 and 18

Day 60 opinion

**Action:** For adoption

Vaccines / Infectious Diseases

2.1.14. [Betrixaban - EMEA-001834-PIP02-16](#)

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Prevention of venous thromboembolism / Adults and children

Day 60 adoption

**Action:** For adoption

Cardiovascular Diseases

2.1.15. [Peramivir - EMEA-001856-PIP02-16](#)

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Treatment of influenza / Treatment of influenza

Day 60 discussion

**Action:** For adoption

Infectious Diseases

## 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. [Gemtuzumab linked to Ozogamicin - EMEA-C1-001733-PIP02-15](#)

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Pfizer Limited; Treatment of acute myeloid leukaemia

Day 60 opinion

**Action:** For adoption

Oncology

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

### 2.3.1. sacubitril / valsartan - EMEA-000316-PIP02-11-M03

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Novartis Europharm Ltd.; Heart failure / Treatment of heart failure

Day 60 opinion

**Action:** For adoption

Cardiovascular Diseases

### 2.3.2. Allantoin - Orphan - EMEA-001590-PIP01-13-M03

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Sciaderm, Inc.; Treatment of epidermolysis bullosa

Day 60 opinion

**Action:** For adoption

Dermatology

### 2.3.3. Liquid extract ethanolic 30 per cent (w/w) of Allium cepa L. (fresh bulb) and Citrus limon (L.) Burm. f. (fresh fruit), Paullinia cupana Kunth, Theobroma cacao L. - EMEA-001835-PIP01-15-M01

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Legacy Healthcare; Treatment of alopecia

Day 60 opinion

**Action:** For adoption

Dermatology

### 2.3.4. Rubidium Rb-82 Chloride - EMEA-000882-PIP03-11-M02

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Jubilant DraxImage Inc.; Visualization of myocardial perfusion for diagnostic purposes

Day 60 opinion

**Action:** For adoption

Diagnostic

### 2.3.5. Estetrol & Drospirenone - EMEA-001332-PIP01-12-M02

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Estetra SPRL; Prevention of pregnancy

Day 60 opinion

**Action:** For adoption

Endocrinology-Gynaecology-Fertility-Metabolism

### 2.3.6. deferiprone - Orphan - EMEA-001126-PIP01-10-M02

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Consorzio per Valutazioni Biologiche e Farmacologiche (CVBF) - Coordinator for DEEP Project (HEALTH-F4-2010-261483); treatment of chronic iron overload requiring chelation therapy / treatment of iron overload in paediatric patients affected by haemoglobinopathies requiring chronic transfusions and iron chelation

Day 60 opinion

**Action:** For adoption

Haematology-Hemostaseology

### 2.3.7. Ceftaroline fosamil - EMEA-000769-PIP01-09-M06

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AstraZeneca AB; Treatment of cSSTI (complicated skin and soft tissue infections), Treatment of CAP (community-acquired pneumonia) / Treatment of cSSTI (complicated skin and soft tissue infections), Treatment of CAP (community-acquired pneumonia)

Day 60 opinion

**Action:** For adoption

Infectious Diseases

### 2.3.8. Talimogene laherparepvec - EMEA-001251-PIP01-11-M02

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Amgen Europe B.V.; Treatment of solid malignant non-CNS tumours

Day 60 opinion

**Action:** For adoption

Oncology

### 2.3.9. Finerenone - EMEA-001623-PIP01-14-M01

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Bayer Pharma AG; Chronic Kidney Disease / Treatment of chronic kidney disease associated with proteinuria in addition to a therapy with ACEi or ARB

Day 60 opinion

**Action:** For adoption

Uro-nephrology

### 2.3.10. Metreleptin - Orphan - EMEA-001701-PIP01-14-M01

---

Aegerion Pharmaceuticals Ltd; Treatment of lipodystrophy

Day 30 adoption

**Action:** For adoption

Endocrinology-Gynaecology-Fertility-Metabolism

## 2.4. Opinions on Re-examinations

No items.

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

---

Vtesse Europe Ltd; Treatment of Niemann-Pick disease, type C

Day 90 discussion

**Action:** For discussion

Endocrinology-Gynaecology-Fertility-Metabolism

### 3.1.2. pegvaliase - Orphan - EMEA-001951-PIP01-16

---

BioMarin International Limited; For the treatment of hyperphenylalaninaemia / For the treatment of hyperphenylalaninaemia in paediatric patients of all ages with phenylketonuria

Day 90 discussion

**Action:** For discussion

Endocrinology-Gynaecology-Fertility-Metabolism

### 3.1.3. Volanesorsen - Orphan - EMEA-001915-PIP01-15

---

Ionis Pharmaceuticals; Familial Chylomicronemia Syndrome

Day 90 discussion

**Action:** For discussion

Endocrinology-Gynaecology-Fertility-Metabolism

### 3.1.4. Autologous CD34+ cells transduced with lentiviral vector encoding the human beta-globin gene - Orphan - EMEA-001933-PIP01-16

---

Fondazione Telethon; Beta-thalassemia major and intermedia / Treatment of Beta thalassemia

Day 90 discussion

**Action:** For discussion

Haematology-Hemostaseology

### 3.1.5. vadadustat - EMEA-001944-PIP01-16

---

Anaemia secondary to chronic kidney disease / Treatment of anaemia secondary to chronic kidney disease

Day 90 discussion

**Action:** For discussion

Haematology-Hemostaseology

### 3.1.6. abatacept - EMEA-000118-PIP03-15

---

Treatment of childhood-onset SLE / Treatment of childhood-onset lupus nephritis caused by childhood-onset SLE with abatacept in combination with MMF or CY, and CS in pediatric patients 5 years of age and older who have had an insufficient response to MMF or CY, and CS.

Day 90 discussion

**Action:** For discussion

Immunology-Rheumatology-Transplantation

### 3.1.7. Ciprofloxacin Hydrochloride - EMEA-001563-PIP02-15

---

Treatment of cystic fibrosis related bronchiectasis associated with P. aeruginosa infection, Treatment of non-cystic fibrosis related bronchiectasis associated with P. aeruginosa infection (NCFBEPA+)

Day 90 discussion

**Action:** For discussion

Infectious Diseases

### 3.1.8. Anti-(human calcitonin gene-related peptide receptor) human monoclonal antibody - EMEA-001664-PIP02-15

---

Migraine headaches / Prophylaxis of migraine

Day 90 discussion

**Action:** For discussion

Neurology

### 3.1.9. EMEA-001428-PIP03-15

---

Major Depressive Disorder (MDD)

Day 90 discussion

**Action:** For discussion

Psychiatry

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

---

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

Day 90 discussion

**Action:** For discussion

Uro-nephrology / Haematology-Hemostaseology

### 3.1.11. Varicella-zoster virus (VZV), Oka/Merck, inactivated, vaccine - EMEA-001073-PIP02-14

---

Prevention of Varicella Zoster Virus disease / Prevention of HZ in immunocompromised patients from 1 to less than 18 years of age

Day 90 discussion

**Action:** For discussion

Vaccines

### 3.1.12. rAAV8-hUGT1A1 - Orphan - EMEA-002021-PIP01-16

---

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

Day 60 discussion

**Action:** For discussion

Endocrinology-Gynaecology-Fertility-Metabolism

### 3.1.13. Filgotinib - EMEA-001619-PIP03-16

---

Ulcerative colitis (UC), Crohn's disease (CD) / Treatment of paediatric patients 2 years of age and older with moderately-to-severely active ulcerative colitis, Treatment of paediatric patients 2 years of age and older with moderately-to-severely active Crohn's disease

Day 60 discussion

**Action:** For discussion

Gastroenterology-Hepatology

3.1.14. [Methyl 3-\(\(2R\)-2-hydroxy-4-\(\(\(\(S\)-1-methoxy-1-oxopropan-2-yl\)amino\)\(phenoxy\)phosphoryl\)oxy\)-3,3-dimethylbutanamido\)propanoate - Orphan - EMEA-002036-PIP01-16](#)

---

Retrophin Europe Limited; Treatment of Pantothenate Kinase Associated Neurodegeneration (PKAN)

Day 60 discussion

**Action:** For discussion

Neurology

3.1.15. [Sarizotan hydrochloride - Orphan - EMEA-001808-PIP02-16](#)

---

Newron Pharmaceuticals SpA; Treatment of Rett syndrome

Day 60 discussion

**Action:** For discussion

Neurology

3.1.16. [Entolimod - Orphan - EMEA-002020-PIP01-16](#)

---

Cleveland BioLabs Inc; Treatment of acute Radiation Syndrome / Entolimod is indicated for reducing the risk of death following exposure to potentially lethal irradiation occurring as the results of a radiation disaster

Day 60 discussion

**Action:** For discussion

Other

3.1.17. [pimavanserin - EMEA-001688-PIPO3-16](#)

---

Treatment of schizophrenia and other psychotic disorders

Day 60 discussion

**Action:** For discussion

Psychiatry

3.1.18. [Apolipoprotein A-1 \(ApoA-1\) - EMEA-002040-PIP01-16](#)

---

Treatment of Acute Myocardial Infarction



Day 30 discussion

**Action:** For discussion

Cardiovascular Diseases

### 3.1.19. [Candesartan cilexetil / Amlodipine besylate / Hydrochlorothiazide - EMEA-002024-PIP01-16](#)

---

Treatment of essential hypertension (ICD9: 401, ICD10: I10)

Day 30 discussion

**Action:** For discussion

Cardiovascular Diseases

### 3.1.20. [tadalafil / ambrisentan - EMEA-002030-PIP01-16](#)

---

Pulmonary Arterial Hypertension

Day 30 discussion

**Action:** For discussion

Cardiovascular Diseases

### 3.1.21. [Ligelizumab - EMEA-001811-PIP02-15](#)

---

Treatment of chronic spontaneous urticaria / Treatment of chronic spontaneous urticaria

Day 30 discussion

**Action:** For discussion

Dermatology

### 3.1.22. [EMEA-002039-PIP01-16](#)

---

Treatment of uterine leiomyoma (fibroids), Treatment of endometriosis / Treatment of uterine fibroids, Treatment of endometriosis

Day 30 discussion

**Action:** For discussion

Endocrinology-Gynaecology-Fertility-Metabolism

### 3.1.23. [Empagliflozin - EMEA-000828-PIP04-16](#)

---

Treatment of type 1 diabetes mellitus

Day 30 discussion

**Action:** For discussion

Endocrinology-Gynaecology-Fertility-Metabolism

3.1.24. Synthetic double-stranded small interfering ribonucleic acid directed against delta-aminolevulinic acid synthase mRNA that is covalently linked to a ligand containing three N-acetylgalactosamine residues - EMEA-002048-PIP01-16

---

Acute Hepatic Porphyria (AHP)

Day 30 discussion

**Action:** For discussion

Endocrinology-Gynaecology-Fertility-Metabolism

3.1.25. Synthetic double-stranded siRNA oligonucleotide directed against hydroxyacid oxidase 1 mRNA that is covalently linked to a ligand containing three N-acetylgalactosamine residues - Orphan - EMEA-002079-PIP01-16

---

Alnylam UK Limited; Treatment of Primary Hyperoxaluria Type 1 / Treatment of Primary Hyperoxaluria Type 1

Day 30 discussion

**Action:** For discussion

Gastroenterology-Hepatology

3.1.26. Plasminogen (human) - Orphan - EMEA-002044-PIP01-16

---

ProMetic BioTherapeutics Ltd; Plasminogen deficiency

Day 30 discussion

**Action:** For discussion

Haematology-Hemostaseology

3.1.27. anifrolumab - EMEA-001435-PIP02-16

---

Lupus nephritis, Systemic lupus erythematosus

Day 30 discussion

**Action:** For discussion

Immunology-Rheumatology-Transplantation

3.1.28. EMEA-001989-PIP01-16

---

Staphylococcal skin infection / Treatment of acute bacterial skin and skin structure infections (ABSSSI) due to staphylococcus sensitive or resistant to meticillin

Day 30 discussion

**Action:** For discussion

Infectious Diseases

3.1.29. [VIS410 \(human immunoglobulin G1 monoclonal antibody directed against a unique, functionally conserved epitope on the influenza A haemagglutinin protein\) - EMEA-001924-PIP01-15](#)

---

Influenza A (ICD10 code: J09) / Treatment of influenza A

Day 30 discussion

**Action:** For discussion

Infectious Diseases

3.1.30. [Doxorubicin hydrochloride - Orphan - EMEA-002043-PIP01-16](#)

---

ONXEO S.A.; Treatment of hepatocellular carcinoma / Treatment of hepatocellular carcinoma

Day 30 discussion

**Action:** For discussion

Oncology

3.1.31. [mirvetuximab soravtansine - Orphan - EMEA-001921-PIP01-16](#)

---

ImmunoGen Europe Limited; For the treatment of ovarian carcinoma (excluding rhabdomyosarcoma and germ cell tumours), For the treatment of peritoneal carcinoma, For the treatment of fallopian tube carcinoma (excluding rhabdomyosarcoma and germ cell tumours)

Day 30 discussion

**Action:** For discussion

Oncology

3.1.32. [Polihexanide \(PHMB\) - Orphan - EMEA-002053-PIP01-16](#)

---

Società Industria Farmaceutica Italiana (S.I.F.I.) SpA; ICD10: B.60.1 Keratitis and keratoconjunctivitis (interstitial) in acanthamoebiasis

Day 30 discussion

**Action:** For discussion

Ophthalmology

3.1.33. [Vosoritide - Orphan - EMEA-002033-PIP01-16](#)

---

BioMarin International Limited; Treatment of achondroplasia / Treatment of achondroplasia

Day 30 discussion

**Action:** For discussion

Other

### 3.1.34. [Formoterol Fumarate / Glycopyrronium Bromide / Budesonide - EMEA-002063-PIP01-16](#)

---

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 30 discussion

**Action:** For discussion

Pneumology - Allergology

### 3.1.35. [EMEA-002062-PIP01-16](#)

---

Interstitial Cystitis/Bladder Pain Syndrome

Day 30 discussion

**Action:** For discussion

Uro-nephrology

## 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. [Catridecacog - EMEA-C-000185-PIP01-08-M05](#)

---

Novo Nordisk A/S; Treatment of congenital factor XIII A-subunit deficiency

Day 30 discussion

**Action:** For discussion

Haematology-Hemostaseology

### 3.2.2. [Damoctocog alfa pegol - EMEA-C1-001229-PIP01-11-M02](#)

---

Bayer Pharma AG; Treatment of hereditary Factor VIII deficiency

Day 30 discussion

**Action:** For discussion

Haematology-Hemostaseology

### 3.2.3. Fibrinogen (human plasma-derived) - EMEA-C-000457-PIP02-10-M02

---

LFB Biotechnologies; Treatment of congenital fibrinogen deficiency

Day 30 discussion

**Action:** For discussion

Haematology-Hemostaseology

### 3.2.4. tenofovir disoproxil fumarate / emtricitabine - EMEA-C-001091-PIP02-15

---

Gilead Sciences International Ltd.; Treatment of Human Immunodeficiency (HIV-1) infection

Day 30 discussion

**Action:** For discussion

Infectious Diseases

### 3.2.5. natalizumab - EMEA-C-001095-PIP02-12

---

Biogen Idec Ltd; Treatment of Multiple Sclerosis

Day 30 discussion

**Action:** For discussion

Neurology

### 3.2.6. Dinutuximab - EMEA-C-001285-PIP01-12-M02

---

United Therapeutics Europe Limited; Treatment of Neuroblastoma

Day 30 discussion

**Action:** For discussion

Oncology

### 3.2.7. Nilotinib - EMEA-C-000290-PIP01-08-M04

---

Novartis Europharm Ltd.; Treatment of chronic myeloid leukaemia

Day 30 discussion

**Action:** For discussion

Oncology

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

#### 3.3.1. edoxaban tosilate - EMEA-000788-PIP02-11-M05

---

Daiichi Sankyo Europe GmbH; 82 Other venous embolism and thrombosis, 174 Arterial embolism and thrombosis, 180 Phlebitis and thrombophlebitis / Prevention of arterial thromboembolism in paediatric cardiac patients at risk of thrombotic events, Acute treatment & secondary prevention of symptomatic recurrent venous thrombotic events (VTE) in paediatric patients at risk

Day 30 discussion

**Action:** For discussion

Cardiovascular Diseases

#### 3.3.2. ticagrelor - EMEA-000480-PIP01-08-M09

---

AstraZeneca AB; thromboembolic events (children), acute coronary syndrome / reduction in occurrence of vaso- occlusive crisis in paediatric patients with sickle cell disease, waiver

Day 30 discussion

**Action:** For discussion

Cardiovascular Diseases / Haematology-Hemostaseology

#### 3.3.3. Terbinafine hydrochloride - EMEA-001259-PIP02-13-M01

---

Polichem SA; Treatment of onychomycosis / Treatment of onychomycosis

Day 30 discussion

**Action:** For discussion

Dermatology

#### 3.3.4. Alirocumab - EMEA-001169-PIP01-11-M02

---

Sanofi-aventis Recherche & Developpement; Prevention of cardiovascular events, Treatment of elevated cholesterol / Treatment of heterozygous and homozygous familial hypercholesterolemia, Proposed adult indication: to reduce the risk of cardiovascular events in adult patients with a history of an acute coronary syndrome.

Day 30 discussion

**Action:** For discussion

Endocrinology-Gynaecology-Fertility-Metabolism

### 3.3.5. [Idursulfase - EMEA-000294-PIP02-12-M01](#)

---

Shire Human Genetic Therapies AB; ICD10 E76.1: / Treatment of Mucopolysaccharidosis II (Hunter Syndrome)

Day 30 discussion

**Action:** For discussion

Endocrinology-Gynaecology-Fertility-Metabolism

### 3.3.6. [Potassium chloride / Sodium chloride / Citric acid, anhydrous / Sodium citrate / Simeticone / Sodium sulphate, anhydrous / Macrogol 4000 - EMEA-001356-PIP02-12-M01](#)

---

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

Day 30 discussion

**Action:** For discussion

Gastroenterology-Hepatology

### 3.3.7. [Eltrombopag \(eltrombopag olamine\) - EMEA-000170-PIP03-13-M02](#)

---

Novartis Europharm Limited; Bone Marrow Depression and Hypoplastic Anaemia / Treatment of cytopenias in paediatric patients with severe aplastic anaemia who are not receiving hematopoietic stem cell transplant

Day 30 discussion

**Action:** For discussion

Haematology-Hemostaseology

### 3.3.8. [Methoxy polyethylene glycol- epoetin beta - EMEA-000172-PIP01-07-M02](#)

---

Roche Registration Limited; Anaemia associated with chronic kidney disease

Day 30 discussion

**Action:** For discussion

Haematology-Hemostaseology

### 3.3.9. [belatacept - EMEA-000157-PIP01-07-M03](#)

---

Bristol-Myers Squibb Pharma EEIG; Prevention of rejection of transplanted kidney / NULOJIX, in combination with corticosteroids and a mycophenolic acid (MPA), is indicated for prophylaxis of graft rejection in pediatric patients at least 12 years of age and with a stable renal transplant for at least 6 months, who convert to a CNI-free maintenance immunosuppressive regimen.

Day 30 discussion

**Action:** For discussion

Immunology-Rheumatology-Transplantation

3.3.10. [4-amino-1-\[5-chloro-2,5-dideoxy-2-fluoro-3-O-\(2-methylpropanoyl\)-4-\[\[2-methylpropanoyl\]oxy\]methyl\]-a-L-lyxofuranosyl\]-2\(1H\)-pyrimidinone - EMEA-001758-PIP01-15-M01](#)

---

Janssen-Cilag International NV; Treatment of lower respiratory tract disease caused by human respiratory syncytial virus

Day 30 discussion

**Action:** For discussion

Infectious Diseases

3.3.11. [bedaquiline \(fumarate\) - Orphan - EMEA-000912-PIP01-10-M03](#)

---

Janssen-Cilag International NV; Treatment of multi-drug resistant tuberculosis / Treatment of multi-drug resistant tuberculosis

Day 30 discussion

**Action:** For discussion

Infectious Diseases

3.3.12. [elbasvir / grazoprevir - EMEA-001604-PIP01-13-M02](#)

---

Merck Sharp & Dohme (Europe), Inc.; treatment of chronic Hepatitis C infection / Treatment of chronic hepatitis C genotype 1, 4, and 6 infection with the combination regimen of MK-5172 and MK-8742 in children and adolescents from 3 years to less than 18 years of age with compensated liver disease who are previously untreated or who have failed previous Peg-Interferon/Interferon therapy with ribavirin with or without cirrhosis.

Day 30 discussion

**Action:** For discussion

Infectious Diseases

3.3.13. [Rilpivirine \(RPV\) / Dolutegravir \(DTG\) - EMEA-001750-PIP01-15-M01](#)

---

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

Day 30 discussion

**Action:** For discussion

Infectious Diseases



### 3.3.14. [Tenofovir Alafenamide / Emtricitabine - EMEA-001577-PIP02-14-M01](#)

---

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

Day 30 discussion

**Action:** For discussion

Infectious Diseases

### 3.3.15. [Amikacin sulfate - Orphan - EMEA-000525-PIP01-08-M05](#)

---

Insmed Limited; Treatment of nontuberculous mycobacterial (NTM) lung infection / Treatment of nontuberculous mycobacterial (NTM) lung infection

Day 30 discussion

**Action:** For discussion

Infectious Diseases / Pneumology - Allergology

### 3.3.16. [Nanobody directed towards the fusion protein of human respiratory syncytial virus - EMEA-001553-PIP01-13-M01](#)

---

Ablynx NV; Lower respiratory tract disease caused by RSV / Treatment of RSV lower respiratory tract infection

Day 30 discussion

**Action:** For discussion

Neonatology - Paediatric Intensive Care

### 3.3.17. [Bumetanide - EMEA-001303-PIP01-12-M01](#)

---

Neurochlore; Autism Spectrum Disorder / Treatment of Autism Spectrum Disorder

Day 30 discussion

**Action:** For discussion

Neurology

### 3.3.18. [Humanized anti-IL-6 receptor \(IL-6R\) monoclonal antibody - Orphan - EMEA-001625-PIP01-14-M01](#)

---

CHUGAI PHARMA EUROPE LTD.; neuromyelitis optica

Day 30 discussion

**Action:** For discussion

Neurology

### 3.3.19. Ocrelizumab - EMEA-000310-PIP03-10-M02

---

Roche Registration Ltd; Multiple Sclerosis / Treatment of Relapsing Remitting Multiple Sclerosis (RRMS)

Day 30 discussion

**Action:** For discussion

Neurology

### 3.3.20. Idelalisib - EMEA-001350-PIP02-13-M03

---

Gilead Sciences International Ltd; Treatment of mature B-cell neoplasm / Treatment of children from 1 year to less than 18 years of age with a relapsed or refractory diffuse large B-cell lymphoma (DLBCL) or mediastinal B-cell lymphoma (MBCL)

Day 30 discussion

**Action:** For discussion

Oncology

### 3.3.21. ipilimumab - EMEA-000117-PIP02-10-M07

---

Bristol-Myers Squibb Pharma EEIG; Treatment of melanoma / Treatment of pre-treated and naive patients with advanced metastatic melanoma.

Day 30 discussion

**Action:** For discussion

Oncology

### 3.3.22. midostaurin - Orphan - EMEA-000780-PIP01-09-M03

---

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

### 3.3.23. nivolumab - EMEA-001407-PIP02-15-M01

---

Bristol-Myers Squibb Pharma EEIG; Treatment of malignant neoplasms of lymphoid tissue, Treatment of malignant neoplasms of the central nervous system / Treatment of paediatric patients with relapsed or refractory Hodgkin lymphoma in the age group from 5 years to < 18 years., Treatment of paediatric patients with a relapsed or refractory non-Hodgkin lymphoma in the age group from 6 months to less than 18 years old., Treatment of paediatric patients from 6 months to less than 18 years of age with a recurrent or

progressive high-grade glioma.

Day 30 discussion

**Action:** For discussion

Oncology

### 3.3.24. paclitaxel - EMEA-001308-PIP01-12-M01

---

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

Day 30 discussion

**Action:** For discussion

Oncology

### 3.3.25. ranibizumab - EMEA-000527-PIP04-13-M01

---

Novartis Europharm Limited; retinopathy of prematurity / Treatment of patients with retinopathy of prematurity

Day 30 discussion

**Action:** For discussion

Ophthalmology

### 3.3.26. Patiromer sorbitex calcium - EMEA-001720-PIP01-14-M01

---

Vifor Fresenius Medical Care Renal Pharma France; Hyperkalaemia

Day 30 discussion

**Action:** For discussion

Other

### 3.3.27. CONCENTRATE OF PROTEOLYTIC ENZYMES ENRICHED IN BROMELAIN - Orphan - EMEA-000142-PIP02-09-M05

---

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

Day 30 discussion

**Action:** For discussion

Other / Dermatology

### 3.3.28. budesonide - EMEA-001087-PIP02-12-M03

---

Vectura Limited; Treatment of asthma

Day 30 discussion

**Action:** For discussion

Pneumology - Allergology

### 3.3.29. Dupilumab - EMEA-001501-PIP02-13-M02

---

sanofi-aventis recherche & développement; Asthma

Day 30 discussion

**Action:** For discussion

Pneumology - Allergology

### 3.3.30. Formoterol fumarate dihydrate / Beclometasone dipropionate - EMEA-000548-PIP01-09-M06

---

Chiesi Farmaceutici S.p.A.; COPD, Asthma / Maintenance therapy of asthma where use of a combination product (inhaled corticosteroid and long-acting beta2-agonist) is appropriate: - patients not adequately controlled with inhaled corticosteroids and 'as needed' inhaled short-acting beta2-agonist or - patients already adequately controlled on both inhaled corticosteroids and long-acting beta2-agonists

Day 30 discussion

**Action:** For discussion

Pneumology - Allergology

### 3.3.31. Neisseria meningitidis serogroup B recombinant lipoprotein (rLP2086; subfamily B; Escherichia coli) / Neisseria meningitidis serogroup B recombinant lipoprotein (rLP2086; subfamily A; Escherichia coli) - EMEA-001037-PIP02-11-M04

---

Pfizer Ltd; Prevention of Invasive meningococcal disease caused by N meningitidis serogroup B

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 January 2017 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. Istradefylline - EMEA-31-2016**

---

Treatment of Parkinson's disease (non-juvenile)/ Treatment of moderate to severe Parkinson's disease

**Action:** For adoption

##### **6.1.2. EMEA-32-2016**

---

The class of Her- / epidermal growth factor-receptor antibody medicinal products for treatment of breast malignant neoplasms, intestinal malignant neoplasms and head and neck epithelial malignant neoplasms

**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**

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. Elections of PDCO Vice-Chair**

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PDCO Chair: Dirk Mentzer

**Action:** For adoption

### **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. Good practice guide (GPG) recommendations for paediatric population**

---

**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: Jacqueline Carleer

**Action:** For information

#### 9.3.2. Formulation Working Group

---

PDCO member: Brian Aylward

**Action:** For information

#### 9.3.3. Formulation Working Group session at PDCO

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PDCO member: Brian Aylward

Delegation attending PDCO

**Action:** For discussion

#### 9.3.4. Finalisation of Post-Authorisation Efficacy Study (PAES) scientific guidance

---

**Action:** For adoption

### 9.4. Cooperation within the EU regulatory network

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

---

**Action:** For discussion

#### 9.4.2. Report from the meeting 'Medicines: policy, authorisation and monitoring' held in Brussels on 19 September 2016

---

**Action:** For information

## 9.5. Cooperation with International Regulators

### 9.5.1. Report on the progress of the update of the EMA/FDA strategic document on Gaucher disease

---

PDCO member: Sylvie Benchetrit

**Action:** For information

### 9.5.2. FDA and SmartTots (Strategies for Mitigating Anesthesia-Related neuroToxicity in Tots) initiative: Defining Safe Use of Anesthesia in Children and interpretation of non-clinical evidence

---

PDCO member: Jacqueline Carleer

**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

### 9.7.1. PDCO Work-plan 2017

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**Action:** For discussion

## 9.8. Planning and reporting

### 9.8.1. Report from the Strategic Review and Learning Meeting (SRLM) held in Brussels on 19-21 October 2016

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PDCO Chair: Dirk Mentzer

**Action:** For information

### 9.8.2. Draft Agenda of the Strategic Review and Learning Meeting (SRLM) to be held in Malta on 12-14 April 2017

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PDCO member: John Joseph Borg

**Action:** For information



## 10. Any other business

## 11. Breakout sessions

### 11.1.1. Paediatric oncology

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**Action:** For discussion on Tuesday, 16:00 - 17:00, room 3J

### 11.1.2. Neonatology

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**Action:** For discussion on Tuesday, 16:00 - 17:00, 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/)