12 September 2016
EMA/PDCO/534561/2016
Inspections, Human Medicines Pharmacovigilance and Committees Division

# Paediatric Committee (PDCO)

## Draft agenda for the meeting on 14-16 September 2016

Chair: Dirk Mentzer – Vice-Chair: Koenraad Norga

14 September 2016, 08:30- 19:00, room 3A

15 September 2016, 08:30- 19:00, room 3A

16 September 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

## 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 14-16 September 2016. See September 2016 PDCO minutes (to be published post PDCO October 2016 meeting).

## 1.2. Adoption of agenda

PDCO agenda for 14-16 September 2016.

## 1.3. Adoption of the minutes

PDCO minutes for 17-19 August 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. Ascorbic Acid / Sodium Ascorbate / Potassium Chloride / Sodium Chloride / Sodium Sulfate / Macrogol 3350 - EMEA-001705-PIP02-15

Diagnosis of large intestine disorders / For bowel cleansing prior to any clinical procedures requiring a clean bowel e.g. bowel endoscopy or radiology

Day 120 opinion

**Action**: For adoption

Gastroenterology-Hepatology

### 2.1.2. Monoclonal IgG1 anti-influenza A antibody - EMEA-001831-PIP01-15

Treatment of influenza / Treatment of patients hospitalised with severe influenza A virus infection

Day 120 opinion

**Action**: For adoption

Infectious Diseases

### 2.1.3. EMEA-001877-PIP01-15

Episodic Migraine, Chronic Migraine / Prophylaxis of headache in children aged 12 to 18 years

with chronic migraine, Prophylaxis of headache in children aged 6 to 18 years with episodic migraine

Day 120 opinion

**Action**: For adoption

Neurology

### 2.1.4. Humanised chimeric antibody with a humanised H chain and a chimeric (mouse V-domain, human C-domain) L chain against epidermal growth factor receptor conjugated to maleimidocaproyl monomethylauristatin F - Orphan - EMEA-001732-PIP02-15

AbbVie Ltd; Treatment of high-grade glioma / Treatment of high-grade glioma

Day 120 opinion

**Action**: For adoption

Oncology

### 2.1.5. Birch pollen extract (Betula verrucosa) - EMEA-001879-PIP01-15

Treatment of allergic rhinitis / rhino-conjunctivitis / Treatment of tree pollen allergic rhinitis and / or conjunctivitis

Day 120 opinion

**Action**: For adoption

Pneumology - Allergology

### 2.1.6. Influenza virus surface antigens (haemagglutinin and neuraminidase) of strain B (Victoria lineage) / Influenza virus surface antigens (haemagglutinin and neuraminidase) of strain B (Yamagata lineage) / Influenza virus surface antigens (haemagglutinin and neuraminidase) of strain A (H3N2) / Influenza virus surface antigens (haemagglutinin and neuraminidase) of strain A (H1N1) - EMEA-001894-PIP01-15

Prevention of influenza

Day 120 opinion

**Action**: For adoption

Vaccines

### 2.1.7. Amlodipine / Candesartan - EMEA-002014-PIP01-16

Hypertension

Day 60 opinion

**Action**: For adoption

Cardiovascular Diseases

### 2.1.8. Amlodipine / Perindopril - EMEA-001968-PIP01-16

Hypertension

Day 60 opinion

**Action**: For adoption

Cardiovascular Diseases

### 2.1.9. Hydrochlorothiazide / Valsartan / Amlodipine - EMEA-002006-PIP01-16

Essential hypertension / Treatment of essential hypertension as substitution therapy in adult patients whose blood pressure is adequately controlled on the combination of amlodipine, valsartan and hydrochlorothiazide (HCT), taken either as three single-component formulations or as a dual-component and a single-component formulation

Day 60 opinion

**Action**: For adoption

Cardiovascular Diseases

### 2.1.10. Atorvastatin / Amlodipine - EMEA-002005-PIP01-16

Treatment of concomitant angina and dyslipidaemia, Prevention of cardiovascular events in hypertensive patients and diabetes mellitus type 2 patients with multiple risk factors for cardiovascular disease, Treatment of concomitant hypertension and dyslipidaemia / Substitution therapy in patients already taking concomitantly amlodipine and atorvastatin mono-products for the management of: Concomitant hypertension and dyslipidaemia, Substitution therapy in patients already taking concomitantly amlodipine and atorvastatin mono-products for the management of: Concomitant angina and dyslipidaemia, Substitution therapy in patients already taking concomitantly amlodipine and atorvastatin mono-products for the management of: Prevention of cardiovascular events in hypertensive patients and diabetes mellitus type 2 patients with multiple risk factors for cardiovascular disease

Day 60 opinion

**Action**: For adoption

Endocrinology-Gynaecology-Fertility-Metabolism / Cardiovascular Diseases

### 2.1.11. Sirukumab - EMEA-001043-PIP02-16

Adults: Giant Cell Arteritis, Children: Paediatric vasculitides /Treatment of vasculitides

Day 60 opinion

**Action**: For adoption

Immunology-Rheumatology-Transplantation

### 2.1.12. PEGPH20 (PEGylated recombinant human hyaluronidase PH20, rHuPH20) - Orphan - EMEA-001883-PIP02-16

Halozyme Inc.; Pancreas cancer

Day 60 opinion

**Action**: For adoption

Oncology

### 2.1.13. Pexidartinib - Orphan - EMEA-001939-PIP02-16

Daiichi Sankyo Europe GmbH; Treatment of benign soft tissue neoplasm

Day 60 opinion

**Action**: For adoption

Oncology

### 2.1.14. Ciclosporin - EMEA-001998-PIP01-16

Dry eye disease/Keratoconjuntivitis Sicca

Day 60 opinion

**Action**: For adoption

Ophthalmology

### 2.1.15. Allogeneic Mesenchymal Precursor Cells (Rexlemestrocel-L) - EMEA-001140-PIP02-15

Disc degeneration disease

Day 60 opinion

**Action**: For adoption

Other

## 2.2. Opinions on Compliance Check

The following compliance checks have been put up for discussion and the members of the PDCO have been invited to comment on issues of possible non-compliance

### 2.2.1. ertugliflozin - EMEA-C1-001533-PIP01-13

MSD (Europe) Inc.; Treatment of type II diabetes mellitus

Day 60 opinion

**Action**: For adoption

Endocrinology-Gynaecology-Fertility-Metabolism

### 2.2.2. exanatide - EMEA-C1-000689-PIP01-09-M06

AstraZeneca AB; Treatment of type 2 diabetes mellitus

Day 60 opinion

**Action**: For adoption

Endocrinology-Gynaecology-Fertility-Metabolism

### 2.2.3. Sofosbuvir / ledipasvir - EMEA-C1-001411-PIP01-12-M03

Gilead Sciences International Ltd.; Treatment of chronic hepatitis C

Day 60 opinion

**Action**: For adoption

Infectious Diseases

### 2.2.4. rufinamide - EMEA-C-000709-PIP01-09-M05

Eisai Limited; Treatment of Lennox-Gastaut Syndrome

Day 60 opinion

**Action**: For adoption

Neurology

### 2.2.5. Tralokinumab - EMEA-C1-000782-PIP01-09-M03

MedImmune Ltd; Treatment of asthma

Day 60 opinion

**Action**: For adoption

Pneumology – Allergology

### 2.2.6. Recombinant Human TriPeptidyl Peptidase 1 (rhTPP1) - Orphan - EMEA-C3-001362-PIP01-12-M03

BioMarin International Limited; Neuronal Ceroid Lipofuscinosis Type 2 (NCL2) / Treatment of Neuronal Ceroid Lipofuscinosis Type 2 (NCL2)

**Action**: For information; compliance report adopted via written procedure on 8 September 2016

Neurology

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

### 2.3.1. Alipogene Tiparvovec - Orphan - EMEA-000292-PIP01-08-M03

uniQure biopharma B.V.; Hyperchylomicronaemia

Day 60 opinion

**Action**: For adoption

Cardiovascular Diseases

### 2.3.2. riociguat - Orphan - EMEA-000718-PIP01-09-M06

Bayer Pharma AG; I27.2 Other secondary pulmonary hypertension, I27.0 Primary pulmonary hypertension / Treatment of drug and toxin-induced pulmonary arterial hypertension, Treatment of pulmonary hypertension with unclear multifactorial mechanisms, Treatment of pulmonary veno-occlusive disease (PVOD) and/or pulmonary capillary hemangiomatosis (PCH), Treatment of pulmonary hypertension due to lung disease and /or hypoxia, Treatment of chronic thromboembolic pulmonary hypertension (CTEPH), Treatment of pulmonary hypertension owing to left heart diseases, Treatment of pulmonary arterial hypertension (PAH)

Day 60 opinion

**Action**: For adoption

Cardiovascular Diseases

### 2.3.3. serelaxin - EMEA-001168-PIP01-11-M03

Novartis Europharm Limited; Treatment of Acute Heart Failure / Treatment of acute heart failure following surgical repair of a congenital heart defect

Day 60 opinion

**Action**: For adoption

Cardiovascular Diseases

### 2.3.4. dabigatran etexilate mesilate - EMEA-000081-PIP01-07-M09

Boehringer Ingelheim International GmbH; Treatment of thromboembolic events, Prevention of thomboembolic events / Treatment of venous thromboembolic events in paediatric patients (secondary venous thrombotic event prevention)

Day 60 opinion

**Action**: For adoption

Cardiovascular Diseases / Haematology-Hemostaseology

### 2.3.5. Sodium zirconium cyclosilicate - EMEA-001539-PIP01-13-M01

ZS Pharma, Inc; Hyperkalaemia / Treatment of Hyperkalaemia

Day 60 opinion

**Action**: For adoption

Endocrinology-Gynaecology-Fertility-Metabolism

### 2.3.6. Tolvaptan - EMEA-001231-PIP02-13-M04

Otsuka Pharmaceutical Europe Ltd.; Polycystic Kidney Disease (PKD), Dilutional hyponatraemia / Treatment of chronic (>48 hours) dilutional hyponatraemia resistant to fluid restriction (i.e., euvolemic and hypervolemic hyponatremia) associated with heart failure, cirrhosis or SIADH, Treatment of progression of ADPKD, Treatment of progression of ARPKD

Day 60 opinion

**Action**: For adoption

Endocrinology-Gynaecology-Fertility-Metabolism

### 2.3.7. Autologous CD34+ haematopoietic stem cells transduced with lentiviral vector encoding the human βA-T87Q-globin gene - Orphan - EMEA-001665-PIP01-14-M01

bluebird bio France; β-thalassaemia

Day 60 opinion

**Action**: For adoption

Haematology-Hemostaseology

### 2.3.8. Human normal immunoglobulin for subcutaneous use - EMEA-000454-PIP01-08-M07

Kedrion S.p.A.; D80-D90 Certain disorders involving the immune mechanism. Primary Immunodeficiency Syndromes / Treatment of Primary Immunodeficiency Syndromes

Day 60 opinion

**Action**: For adoption

Immunology-Rheumatology-Transplantation / Haematology-Hemostaseology

### 2.3.9. ataluren - Orphan - EMEA-000115-PIP01-07-M08

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

Day 60 opinion

**Action**: For adoption

Neurology

### 2.3.10. eteplirsen - Orphan - EMEA-001722-PIP01-14-M01

Sarepta International C.V.; Duchenne muscular dystrophy

Day 60 opinion

**Action**: For adoption

Neurology

### 2.3.11. Olaratumab - Orphan - EMEA-001760-PIP01-15-M01

Eli Lilly and Company Limited; Treatment of Soft Tissue Sarcoma, Treatment of Osteosarcoma / Treatment of recurrent rhabdomyosarcoma in children aged from birth to less than 18 years in combination with a standard-of-care chemotherapy regimen, First-line treatment of osteosarcoma in children aged from 5 to 18 years in combination with a standard-of-care chemotherapy regimen.

Day 60 opinion

**Action**: For adoption

Oncology

### 2.3.12. Cenegermin - Orphan - EMEA-001729-PIP01-14-M01

Dompé farmaceutici S.p.A.; Neurotrophic Keratitis

Day 60 opinion

**Action**: For adoption

Ophthalmology

### 2.3.13. Xylitol / Procaine hydrochloride / Magnesium sulphate heptahydrate / Potassium chloride - EMEA-001171-PIP01-11-M01

MIT Gesundheit GmbH; Cardioplegia / Induction of immediate and prolonged diastolic cardiac arrest in open heart surgery

Day 60 opinion

**Action**: For adoption

Other

### 2.3.14. Human thrombin / Human fibrinogen - EMEA-001340-PIP01-12-M02

ProFibrix BV (Mallinckrodt Pharmaceuticals); Treatment of haemorrhage resulting from a surgical procedure / Supportive treatment where standard surgical techniques are insufficient for improvement of haemostasis

Day 60 opinion

**Action**: For adoption

Other / Haematology-Hemostaseology

### 2.3.15. ataluren - Orphan - EMEA-000115-PIP02-09-M03

PTC Therapeutics International, Limited; Cystic Fibrosis ICD10: E84.9 Cystic fibrosis, unspecified / Treatment of cystic fibrosis

Day 60 opinion

**Action**: For adoption

Pneumology - Allergology

### 2.3.16. mepolizumab - Orphan - EMEA-000069-PIP04-13-M01

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

### 2.3.17. mirabegron - EMEA-000597-PIP02-10-M05

Astellas Pharma Europe B.V.; Treatment of idiopathic overactive bladder / Symptomatic treatment of urge incontinence and/or increased urinary frequency and urgency as may occur in patients with overactive bladder syndrome

Day 60 opinion

**Action**: For adoption

Uro-nephrology

### 2.3.18. mirabegron - EMEA-000597-PIP03-15-M02

Astellas Pharma Europe B.V.; Treatment of neurogenic detrusor overactivity / Treatment of detrusor overactivity in children and adolescents with neurogenic bladder dysfunction

Day 60 opinion

**Action**: For adoption

Uro-nephrology

## 2.4. Opinions on Re-examinations

### 2.4.1. Angiotensin II - EMEA-001912-PIP01-15

La Jolla Pharmaceutical Company, Inc.; Treatment of Catecholamine-resistant hypotension associated with distributive shock

Day 30 opinion

**Action**: For adoption

Other

### 2.4.2. Linaclotide - EMEA-000927-PIP01-10-M03

Allergan Pharmaceuticals International Limited; Functional Constipation / in children

Day 30 opinion

**Action**: For adoption

Gastroenterology-Hepatology

## 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. alvimopan - EMEA-001922-PIP01-15

Prevention of postoperative ileus

Day 90 discussion

**Action**: For discussion

Gastroenterology-Hepatology

### 3.1.2. Naldemedine Tosylate - EMEA-001893-PIP01-15

Opioid-induced constipation (OIC)

Day 90 discussion

**Action**: For discussion

Gastroenterology-Hepatology

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

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 90 discussion

**Action**: For discussion

Haematology-Hemostaseology

### 3.1.4. Recombinant Fusion Protein Linking Coagulation Factor VIIa with Albumin (rVIIa-FP) - Orphan - EMEA-001886-PIP01-15

CSL Behring GmbH; Treatment of congenital Haemophilia A or B

Day 90 discussion

**Action**: For discussion

Haematology-Hemostaseology

### 3.1.5. Recombinant Fusion Protein Linking Coagulation Factor VIIa with Albumin (rVIIa-FP) - Orphan - EMEA-001886-PIP02-15

CSL Behring GmbH; Treatment of congenital Factor VII Deficiency

Day 90 discussion

**Action**: For discussion

Haematology-Hemostaseology

### 3.1.6. Galcanezumab - EMEA-001860-PIP03-16

Prophylactic treatment of migraine headache

Day 90 discussion

**Action**: For discussion

Neurology

### 3.1.7. inebilizumab - EMEA-001911-PIP01-15

Treatment of Neuromyelitis Optica (NMO) and Neuromyelitis Optica Spectrum Disorders (NMOSD)

Day 90 discussion

**Action**: For discussion

Neurology

### 3.1.8. EMEA-001983-PIP01-16

Monitoring of renal function

Day 60 discussion

**Action**: For discussion

Diagnostic / Uro-nephrology

### 3.1.9. triheptanoin - Orphan - EMEA-001920-PIP02-16

Ultragenyx Pharmaceutical Inc.; Mitochondrial trifunctional protein (TFP) deficiency, Long-chain 3 hydroxyacyl-CoA dehydrogenase (LCHAD) deficiency, Carnitine palmitoyl transferase 2 (CPT-II) deficiency, Very long-chain acyl-CoA dehydrogenase (VLCAD) deficiency

Day 60 discussion

**Action**: For discussion

Endocrinology-Gynaecology-Fertility-Metabolism

### 3.1.10. Amiselimod - EMEA-001991-PIP01-16

Ulcerative colitis / Treatment of moderately to severely active ulcerative colitis

Day 60 discussion

**Action**: For discussion

Gastroenterology-Hepatology

### 3.1.11. Cenicriviroc mesylate - EMEA-001999-PIP01-16

Treatment of non-alcoholic steatohepatitis (NASH) in subjects with liver fibrosis

Day 60 discussion

**Action**: For discussion

Gastroenterology-Hepatology

### 3.1.12. Human fibrinogen concentrate - EMEA-001931-PIP01-16

Treatment of congenital fibrinogen deficiency

Day 60 discussion

**Action**: For discussion

Haematology-Hemostaseology

### 3.1.13. tazobactam / ceftolozane - EMEA-001142-PIP02-16

Treatment of abdominal and gastrointestinal infections, Treatment of urinary tract infections, Treatment of pneumonia / Treatment of nosocomial pneumonia, Treatment of complicated intra-abdominal infections (cIAI). Please refer to EMA decision (P/0126/2014) in relation to procedure EMEA-001142-PIP-01-11-M01., Treatment of complicated urinary tract infections (cUTI). Please refer to EMA decision (P/0126/2014) in relation to procedure EMEA-001142-PIP-01-11-M01.

Day 60 discussion

**Action**: For discussion

Infectious Diseases

### 3.1.14. acalabrutinib - Orphan - EMEA-001796-PIP03-16

ACERTA PHARMA, BV; Treatment of mature B cell neoplasms / Treatment of children from 1 to < 18 years of age with relapsed/refractory mature B-cell neoplasms (e.g. diffuse large B-cell lymphoma [DLBCL], Burkitt lymphoma [BL] and primary mediastinal B-cell lymphoma [PMBCL]).

Day 60 discussion

**Action**: For discussion

Oncology

### 3.1.15. triheptanoin - Orphan - EMEA-001920-PIP01-15

Ultragenyx Pharmaceutical Inc.; glucose transporter type-1 deficiency syndrome

Day 60 discussion

**Action**: For discussion

Other

### 3.1.16. Orphan - EMEA-001984-PIP01-16

Retrophin Europe Limited; Treatment of Focal Segmental Glomerulosclerosis (FSGS) / Treatment of Focal Segmental Glomerulosclerosis (FSGS)

Day 60 discussion

**Action**: For discussion

Uro-nephrology

### 3.1.17. EMEA-001978-PIP01-16

Hypertension

Day 30 discussion

**Action**: For discussion

Cardiovascular Diseases

### 3.1.18. Isopropyl Alcohol / Chlorhexidine Gluconate - EMEA-002011-PIP01-16

Prevention of infections associated with transcutaneous procedures

Day 30 discussion

**Action**: For discussion

Dermatology

### 3.1.19. Macimorelin - EMEA-001988-PIP01-16

Growth hormone deficiency / Diagnosis of growth hormone deficiency

Day 30 discussion

**Action**: For discussion

Endocrinology-Gynaecology-Fertility-Metabolism / Diagnostic

### 3.1.20. Orphan - EMEA-002023-PIP01-16

ChemoCentryx, Ltd.; Treatment of ANCA-associated vasculitis

Day 30 discussion

**Action**: For discussion

Immunology-Rheumatology-Transplantation

### 3.1.21. Atacicept - EMEA-002004-PIP01-16

Treatment of systemic lupus erythematosus

Day 30 discussion

**Action**: For discussion

Immunology-Rheumatology-Transplantation

### 3.1.22. Recombinant humanised monoclonal antibody against human complement component C5a - EMEA-002009-PIP01-16

Treatment of acute Graft-versus-Host Disease

Day 30 discussion

**Action**: For discussion

Immunology-Rheumatology-Transplantation

### 3.1.23. Synthetic double-stranded siRNA oligonucleotide directed against p53 mRNA - Orphan - EMEA-001993-PIP01-16

Quark Pharmaceuticals Inc.; Prevention of delayed graft function (DGF) after kidney transplantation / Prevention of DGF after transplantation of kidneys from deceased donors ≥ 45 years old

Day 30 discussion

**Action**: For discussion

Immunology-Rheumatology-Transplantation

### 3.1.24. Terguride hydrogenmaleate - Orphan - EMEA-002015-PIP01-16

medac Gesellschaft für klinische Spezialpräparate mbH; Treatment of Systemic scleroderma

Day 30 discussion

**Action**: For discussion

Immunology-Rheumatology-Transplantation

### 3.1.25. Orphan - EMEA-001960-PIP02-16

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

Day 30 discussion

**Action**: For discussion

Neurology

### 3.1.26. EMEA-001877-PIP02-16

Cluster Headache / Not applicable

Day 30 discussion

**Action**: For discussion

Neurology

### 3.1.27. Daunorubicin (liposomal combination) / Cytarabine (liposomal combination) - Orphan - EMEA-001858-PIP02-16

Celator (UK) Ltd; Acute myeloid leukemia / Treatment

Day 30 discussion

**Action**: For discussion

Oncology

### 3.1.28. lenadogene nolparvovec - Orphan - EMEA-001992-PIP02-16

GENSIGHT-BIOLOGICS; Leber Hereditary Optic Neuropathy (LHON)

Day 30 discussion

**Action**: For discussion

Ophthalmology

### 3.1.29. Teprotumumab - EMEA-001973-PIP01-16

Active thyroid eye disease

Day 30 discussion

**Action**: For discussion

Ophthalmology

### 3.1.30. Mexiletine hydrochloride - Orphan - EMEA-002012-PIP01-16

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

Day 30 discussion

**Action**: For discussion

Other

### 3.1.31. EMEA-001742-PIP02-16

Prevention of psychosis / prevention of first episode of psychosis (FEP) in individuals with attenuated psychotic syndrome (APS)

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. dupilumab - EMEA-C1-001501-PIP01-13-M03

Regeneron Pharmaceuticals, Inc.; Treatment of atopic dermatitis

Day 30 discussion

**Action**: For discussion

Dermatology

### 3.2.2. Secukinumab - EMEA-C2-000380-PIP02-09-M03

Novartis Europharm Ltd; Treatment of chronic idiopathic arthritis (including rheumatoid arthritis, spondylarthritis, psoriatic arthritis and juvenile idiopathic arthritis)

Day 30 discussion

**Action**: For discussion

Immunology-Rheumatology-Transplantation

### 3.2.3. Adalimumab - EMEA-C-000366-PIP05-12-M02

AbbVie Ltd; Treatment of non-infectious uveitis

Day 30 discussion

**Action**: For discussion

Immunology-Rheumatology-Transplantation / Ophthalmology / Dermatology / Gastroenterology-Hepatology

### 3.2.4. IvacaftorLumacaftor EMEA-C2-001582-PIP01-13

Vertex Pharmaceuticals (Europe) Limited; Treatment of cystic fibrosis

Day 30 discussion

**Action**: For discussion

Other

### 3.2.5. lisdexamfetamine (dimesylate) - EMEA-C-000553-PIP01-09-M04

Shire Pharmaceutical Contracts Ltd; Treatment of attention Deficit Hyperactivity Disorder (ADHD)

Day 30 discussion

**Action**: For discussion

Psychiatry

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

### 3.3.1. ambrisentan - Orphan - EMEA-000434-PIP01-08-M04

Glaxo Group Limited; Treatment of Pulmonary Arterial Hypertension / Idiopathic (IPAH) and Familial (FPAH) Pulmonary Hypertension; Associated Pulmonary Hypertension (APAH)

Day 30 discussion

**Action**: For discussion

Cardiovascular Diseases

### 3.3.2. Dobutamine - EMEA-001262-PIP01-12-M02

Proveca Limited; Circulatory impairment / haemodynamic insufficiency

Day 30 discussion

**Action**: For discussion

Cardiovascular Diseases

### 3.3.3. Empagliflozin - EMEA-000828-PIP01-09-M05

Boehringer Ingelheim International GmbH; Treatment of type 2 diabetes mellitus

Day 30 discussion

**Action**: For discussion

Endocrinology-Gynaecology-Fertility-Metabolism

### 3.3.4. linagliptin (as base) - EMEA-000498-PIP01-08-M06

Boehringer Ingelheim International GmbH; Type 2 Diabetes Mellitus / Type 2 Diabetes Mellitus

Day 30 discussion

**Action**: For discussion

Endocrinology-Gynaecology-Fertility-Metabolism

### 3.3.5. migalstat hydrochloride - Orphan - EMEA-001194-PIP01-11-M02

Amicus Therapeutics UK Ltd; Fabry disease

Day 30 discussion

**Action**: For discussion

Endocrinology-Gynaecology-Fertility-Metabolism

### 3.3.6. Semaglutide - EMEA-001441-PIP01-13-M01

Novo Nordisk A/S; Diabetes Mellitus type 2 / Treatment of Diabetes Mellitus type 2

Day 30 discussion

**Action**: For discussion

Endocrinology-Gynaecology-Fertility-Metabolism

### 3.3.7. Ceftobiprole medocaril sodium - EMEA-000205-PIP02-11-M02

Basilea Pharmaceutica International Ltd.; J15: Bacterial pneumoniae no elsewhere classified, J13: Pneumonia due to Streptococcus pneumoniae, J14: Pneumonia due to Hemophilus influenzae / Treatment of nosocomial pneumonia, Treatment of community acquired pneumonia

Day 30 discussion

**Action**: For discussion

Infectious Diseases

### 3.3.8. daclatasvir - EMEA-001191-PIP01-11-M02

Bristol-Myers Squibb Pharma EEIG; Treatment of chronic viral hepatitis C / indicated in combination with sofosbuvir (SOF) for the treatment of CHC in children 3 years of age and older, and adolescents.

Day 30 discussion

**Action**: For discussion

Infectious Diseases

### 3.3.9. Eravacycline - EMEA-001555-PIP01-13-M02

Tetraphase Pharmaceuticals, Inc.; Complicated Intra-Abdominal Infection, Complicated Urinary Tract Infection / Complicated Intra-Abdominal Infection, Urinary Tract Infection

Day 30 discussion

**Action**: For discussion

Infectious Diseases

### 3.3.10. Telavancin hydrochloride - EMEA-000239-PIP01-08-M02

Clinigen Healthcare Ltd; Nosocomial Pneumonia (NP), Complicated skin and soft tissue infections (cSSTI) / Waiver, Nosocomial Pneumonia (NP)

Day 30 discussion

**Action**: For discussion

Infectious Diseases

### 3.3.11. Tenofovir alafenamide / Emtricitabine / Bictegravir - EMEA-001766-PIP01-15-M01

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

Day 30 discussion

**Action**: For discussion

Infectious Diseases

### 3.3.12. tenofovir disoproxil / emtricitabine / cobicistat / elvitegravir - EMEA-000970-PIP01-10-M01

Gilead Sciences International Ltd; B23 Human immunodeficiency virus disease [HIV] resulting in other conditions / indicated for the treatment of HIV-1 infection in paediatric patients aged 12 years and over

Day 30 discussion

**Action**: For discussion

Infectious Diseases

### 3.3.13. 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-M01

bluebird bio France; Treatment of adrenoleukodystrophy

Day 30 discussion

**Action**: For discussion

Neurology

### 3.3.14. Delta-9-tetrahydrocannabinol / Cannabidiol - EMEA-000181-PIP01-08-M03

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

Day 30 discussion

**Action**: For discussion

Neurology

### 3.3.15. Perampanel - EMEA-000467-PIP01-08-M08

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 30 discussion

**Action**: For discussion

Neurology

### 3.3.16. Autologous T cells transduced with lentiviral vector containing a chimeric antigen receptor directed against CD19 - Orphan - EMEA-001654-PIP01-14-M01

Novartis Europharm Limited; B cell acute lymphoblastic leukaemia (ALL) / Treatment of B cell acute lymphoblastic leukaemia (ALL) in paediatric patients who have relapsed after at least two prior regimens or are refractory

Day 30 discussion

**Action**: For discussion

Oncology

### 3.3.17. Bosutinib - Orphan - EMEA-000727-PIP01-09-M02

Pfizer Limited; CML / Treatment of CML in children and adolescents (from 1 to <18 years of age) with resistance or intolerance to prior TKI therapy

Day 30 discussion

**Action**: For discussion

Oncology

### 3.3.18. Eribulin - EMEA-001261-PIP01-11-M03

Eisai Europe Ltd; Soft Tissue Sarcoma / Treatment of non-Rhabdomyosarcoma soft tissue sarcoma, Treatment of Rhabdomyosarcoma

Day 30 discussion

**Action**: For discussion

Oncology

### 3.3.19. pixantrone - EMEA-000713-PIP02-10-M04

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

### 3.3.20. Sunitinib - EMEA-000342-PIP01-08-M05

Pfizer Limited; CD10 code C49.4 malignant neoplasms of connective and soft tissue of abdomen - gastro-intestinal stromal tumours (GIST) / Treatment of gastro-intestinal stromal tumour in paediatric patients aged 6 to less than 18

Day 30 discussion

**Action**: For discussion

Oncology

### 3.3.21. Inotuzumab ozogamicin - Orphan - EMEA-001429-PIP01-13-M01

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

Day 30 discussion

**Action**: For discussion

Oncology / Haematology-Hemostaseology

### 3.3.22. Bupropion HCl / Naltrexone HCl - EMEA-001373-PIP01-12-M02

Orexigen Therapeutics Ireland Limited; Treatment of obesity / Treatment of obesity

Day 30 discussion

**Action**: For discussion

Other

### 3.3.23. fentanyl hydrocholoride - EMEA-001509-PIP01-13-M01

Incline Therapeutics Europe Ltd. (a wholly owned subsidiary of The Medicines Company); Treatment of acute pain

Day 30 discussion

**Action**: For discussion

Pain

### 3.3.24. methoxyflurane - EMEA-000334-PIP01-08-M05

Medical Developments UK Ltd; treatment of acute pain / 1. Self administration to conscious patients with minor trauma and associated pain, under supervision of personnel trained in its use 2. For the management of acute pain associated with short surgical procedures, such as the change of dressings, dislocations and injections

Day 30 discussion

**Action**: For discussion

Pain

### 3.3.25. Tapentadol - EMEA-000018-PIP01-07-M12

Grünenthal GmbH; Acute pain / Treatment of acute pain

Day 30 discussion

**Action**: For discussion

Pain

### 3.3.26. Tapentadol - EMEA-000325-PIP01-08-M06

Grünenthal GmbH; Chronic pain / Treatment of chronic pain

Day 30 discussion

**Action**: For discussion

Pain

### 3.3.27. Loxapine - EMEA-001115-PIP01-10-M05

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 30 discussion

**Action**: For discussion

Psychiatry

## 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 3 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. Bevacizumab - EMEA-27-2016

Treatment of mesothelioma / Bevacizumab in combination with pemetrexed and cisplatin is indicated for the first-line treatment of adult patients with unresectable malignant pleural mesothelioma

**Action**: For adoption

# 7. Discussion on the inclusion of 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 Chair

**Action**: For adoption

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

None

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

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

PDCO member: Jacqueline Carleer

**Action**: For information

### 9.3.2. Formulation Working Group

PDCO member: Brian Aylward

**Action**: For information

### 9.3.3. Inventory of paediatric therapeutic needs - Respiratory

**Action**: For adoption

### 9.3.4. Concept paper on the revision of the Guideline on the role of pharmacokinetics in the development of medicinal products in the paediatric population

**Action**: For discussion

### 9.3.5. Report on the responses from the Vaccines Working Party (VWP) on Dengue vaccine PIP

PDCO member: Marta Granstrom

**Action**: For information

### 9.3.6. Report of a joint EMA workshop with patient and healthcare professional representatives about communication on medicines held on 8 March 2016

**Action**: Document tabled for information

### 9.3.7. Minutes of the EMA Human Scientific Committees' Working Parties with Patients' and Consumers' Organisations (PCWP) and Healthcare Professionals' Organisations (HCPWP) joint meeting held on 9 March 2016

**Action**: Document tabled for information

### 9.3.8. Agenda of the EMA Human Scientific Committees' Working Parties with Patients' and Consumers' Organisations (PCWP) and Healthcare Professionals' Organisations (HCPWP) joint meeting – Workshop on social media held on 19 September 2016

**Action**: Document tabled for information

### 9.3.9. Draft Agenda of the EMA Human Scientific Committees' Working Parties with Patients' and Consumers' Organisations (PCWP) and Healthcare Professionals' Organisations (HCPWP) joint meeting to be held on 20 September 2016

**Action**: Document tabled for information

### 9.3.10. Agenda and Minutes of the EMA Human Scientific Committees' Working Parties with Patients' and Consumers' Organisations (PCWP) meeting held on 14 June 2016

**Action**: Documents tabled for information

### 9.3.11. Agenda of the EMA Human Scientific Committees' Working Parties with Patients' and Consumers' Organisations (PCWP) 10th Anniversary meeting held on 14 June 2016

**Action**: Document tabled for information

### 9.3.12. Agenda and Minutes of the EMA Human Scientific Committees' Working Parties with Healthcare Professionals' Organisations (HCPWP) meeting held on 15 June 2016

**Action**: Documents tabled for information

## 9.4. Cooperation within the EU regulatory network

### 9.4.1. European Commission (EC) launches call for expressions of interest for the EMA PDCO Committee: civil society representatives

**Action**: For information (news item on EC website)

## 9.5. Cooperation with International Regulators

### 9.5.1. Addendum (R1) to International Council for Harmonisation (ICH E11) Guideline 'Clinical Investigation of Medicinal Products in Paediatric Population'

PDCO Chair: Dirk Mentzer

**Action**: For adoption prior to public consultation

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

### 9.6.1. International Neonatal Consortium

**Action**: For discussion

## 9.7. PDCO work plan

### 9.7.1. PDCO Work Plan 2017

**Action**: For discussion

## 9.8. Planning and reporting

None

## 9.9. PDCO ORGAM

None

# 10. Any other business

### 10.1.1. EMA – internal organisational adjustments

**Action**: For information

### 10.1.2. Business Pipeline Report Q3 2016

**Action**: Document tabled for information

# 11. Breakout sessions

### 11.1.1. Paediatric oncology

**Action**: For discussion on Thursday, 18:00 – 19:00, room 3M

### 11.1.2. Neonatology

**Action**: For discussion on Thursday, 18:00 – 19:00, room 3L

## 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/