26 June 2018
EMA/376005/2018 Corr.1
Inspections, Human Medicines Pharmacovigilance and Committees Division

Paediatric Committee (PDCO)

Draft agenda for the meeting on 26-29 June 2018

Chair: Dirk Mentzer – Vice-Chair: Koenraad Norga

26 June 2018, 14:00- 19:00, room 3A

27 June 2018, 08:30- 19:00, room 3A

28 June 2018, 08:30- 19:00, room 3A

29 June 2018, 08:30- 13:00, room 3A

Health and safety information

In accordance with the Agency's health and safety policy, delegates are to be briefed on health, safety and emergency information and procedures prior to the start of the meeting.

Disclaimers

Some of the information contained in this agenda is considered commercially confidential or sensitive and therefore not disclosed. With regard to intended therapeutic indications or procedure scopes listed against products, it must be noted that these may not reflect the full wording proposed by applicants and may also vary during the course of the review. Additional details on some of these procedures will be published in the PDCO Committee meeting reports (after the PDCO Opinion is adopted), and on the Opinions and decisions on paediatric investigation plans webpage (after the EMA Decision is issued).

Note on access to documents

Some documents mentioned in the agenda cannot be released at present following a request for access to documents within the framework of Regulation (EC) No 1049/2001 as they are subject to on-going procedures for which a final decision has not yet been adopted. They will become public when adopted or considered public according to the principles stated in the Agency policy on access to documents (EMA/127362/2006).

30 Churchill Place ● Canary Wharf ● London E14 5EU ● United Kingdom
Telephone +44 (0)20 3660 6000 **Facsimile** +44 (0)20 3660 5555
Send a question via our website www.ema.europa.eu/contact

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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 26-29 June 2018. See June 2018 PDCO minutes (to be published post July 2018 PDCO meeting).

1.2. Adoption of agenda

PDCO agenda for 26-29 June 2018.

1.3. Adoption of the minutes

PDCO minutes for 29 May – 01 June 2018.

2. Opinions

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

2.1. Opinions on Products

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

Wilson Therapeutics AB; Treatment of Wilson disease

Day 120 opinion

Action: For adoption

Endocrinology-Gynaecology-Fertility-Metabolism

2.1.2. EMEA-002287-PIP01-17

Treatment of Type 2 Diabetes Mellitus

Day 120 opinion

Action: For adoption

Endocrinology-Gynaecology-Fertility-Metabolism

2.1.3. Alicaforsen - Orphan - EMEA-002060-PIP02-17

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

Day 120 opinion

Action: For adoption

Gastroenterology-Hepatology

2.1.4. Risankizumab - EMEA-001776-PIP03-17

Crohn's Disease

Day 120 opinion

Action: For adoption

Gastroenterology-Hepatology

2.1.5. Risankizumab - EMEA-001776-PIP04-17

Ulcerative Colitis

Day 120 opinion

Action: For adoption

Gastroenterology-Hepatology

2.1.6. Cefiderocol - EMEA-002133-PIP01-17

Treatment of Gram-negative bacterial infections

Day 120 opinion

Action: For adoption

Infectious Diseases

2.1.7. Glasdegib maleate - Orphan - EMEA-002199-PIP01-17

Pfizer Limited; Treatment of acute myeloid leukaemia (AML)

Day 120 opinion

Action: For adoption

Oncology

2.1.8. 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 120 opinion

Action: For adoption

Oncology

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

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

Day 120 opinion

Action: For adoption

Other

2.1.10. Ferric Pyrophosphate Citrate - EMEA-002261-PIP01-17

Treatment of iron deficient anaemia in haemodialysis patients

Day 120 opinion

Action: For adoption

Uro-nephrology / Haematology-Hemostaseology

2.1.11. 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 120 opinion

Action: For adoption

Vaccines / Infectious Diseases

2.1.12. Clade C gp140 - EMEA-002221-PIP01-17

Prevention of HIV-1 infection in children from 9 to less than 18 years of age

Day 120 opinion

Action: For adoption

Vaccines / Infectious Diseases

2.1.13. Mosaic gp140 - EMEA-002161-PIP01-17

Prevention of HIV-1 infection in children from 9 to less than 18 years of age

Day 120 opinion

Action: For adoption

Vaccines / Infectious Diseases

2.1.14. Amlodipine / irbesartan - EMEA-002352-PIP01-18

Treatment of essential hypertension

Day 60 opinion

Action: For adoption

Cardiovascular Diseases

2.1.15. 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 60 opinion

Action: For adoption

Endocrinology-Gynaecology-Fertility-Metabolism / Uro-nephrology

2.1.16. Luspatercept - EMEA-001521-PIP02-18

Treatment of Myelofibrosis

Day 60 opinion

Action: For adoption

Haematology-Hemostaseology

2.1.17. 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 60 opinion

Action: For adoption

Oncology

2.1.18. Navitoclax - EMEA-000478-PIP02-18

Treatment of myelofibrosis

Day 60 opinion

Action: For adoption

Oncology

2.1.19. Veliparib - Orphan - EMEA-000499-PIP05-18

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

Day 60 opinion

Action: For adoption

Oncology

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

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

Day 60 opinion

Action: For adoption

Haematology-Hemostaseology

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

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

Day 60 letter

Action: For adoption

Infectious Diseases

2.2.3. Quizartinib - EMEA-C1-001821-PIP01-15-M01

Daiichi Sankyo Europe GmbH; Treatment of acute myeloid leukaemia

Day 60 letter

Action: For adoption

Oncology

2.3. Opinions on Modification of an Agreed Paediatric Investigation Plan

2.3.1. Landiolol hydrochloride - EMEA-001150-PIP02-13-M02

AOP Orphan Pharmaceuticals AG; Treatment of supraventricular arrythmias

Day 60 opinion

Action: For adoption

Cardiovascular Diseases

2.3.2. Tralokinumab - EMEA-001900-PIP02-17-M01

LEO Pharma A/S; Treatment of Atopic Dermatitis

Day 60 opinion

Action: For adoption

Dermatology

2.3.3. 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 60 opinion

Action: For adoption

Endocrinology-Gynaecology-Fertility-Metabolism

2.3.4. 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 yers and older

Day 60 opinion

Action: For adoption

Endocrinology-Gynaecology-Fertility-Metabolism

2.3.5. Romosozumab - EMEA-001075-PIP04-15-M01

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

Day 60 opinion

Action: For adoption

Endocrinology-Gynaecology-Fertility-Metabolism

2.3.6. Roxadustat - EMEA-001557-PIP01-13-M02

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

Day 60 opinion

Action: For adoption

Haematology-Hemostaseology

2.3.7. Ciprofloxacin Hydrochloride - EMEA-001563-PIP02-15-M01

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

Day 60 opinion

Action: For adoption

Infectious Diseases

2.3.8. Balovaptan - EMEA-001918-PIP01-15-M01

Roche Registration Ltd; ICD1O 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 60 opinion

Action: For adoption

Neurology

2.3.9. (Z)-N-(3-bromo-4-fluorophenyl)-N'-hydroxy-4-(2-(sulfamoylamino)ethylamino)-1,2,5-oxadiazole-3-carboximidamide - EMEA-002072-PIP01-16-M01

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 60 opinion

Action: For adoption

Oncology

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

Merck KGaA; Treatment of all conditions included in the category of malignant neoplasms (except central nervous system tumours, haematopoietic and lymphoid tissue neoplasm), 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 60 opinion

Action: For adoption

Oncology

2.3.11. Eribulin - EMEA-001261-PIP01-11-M05

Eisai Europe Ltd; Soft Tissue Sarcoma

Day 60 opinion

Action: For adoption

Oncology

2.3.12. Paclitaxel - EMEA-001308-PIP01-12-M02

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

Day 60 opinion

Action: For adoption

Oncology

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

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 60 opinion

Action: For adoption

Oncology

2.3.14. Andexanet alfa - EMEA-001902-PIP01-15-M03

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 60 opinion

Action: For adoption

Other

2.3.15. Febuxostat - EMEA-001417-PIP01-12-M04

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 Sindrome (TLS) affected by hematologic malignancies

Day 60 opinion

Action: For adoption

Other / Oncology

2.3.16. Benralizumab - EMEA-001214-PIP01-11-M08

AstraZeneca AB; Treatment of asthma

Day 60 opinion

Action: For adoption

Pneumology - Allergology

2.3.17. Vortioxetine - EMEA-000455-PIP02-10-M04

H. Lundbeck A/S; Major Depressive Disorder

Day 60 opinion

Action: For adoption

Psychiatry

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

Alexion Europe SAS; Treatment of atypical Haemolytic Uremic Syndrome

Day 60 opinion

Action: For adoption

Uro-nephrology / Haematology-Hemostaseology

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

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

Day 60 opinion

Action: For adoption

Vaccines

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

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

Day 60 opinion

Action: For adoption

Vaccines

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. 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 information

Immunology-Rheumatology-Transplantation

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. Baricitinib - EMEA-001220-PIP03-16

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

Day 90 discussion

Action: For discussion

Dermatology

3.1.2. EMEA-002208-PIP01-17

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

Day 90 discussion

Action: For discussion

Dermatology / Gastroenterology-Hepatology

3.1.3. Inclisiran sodium - EMEA-002214-PIP01-17

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

Day 90 discussion

Action: For discussion

Endocrinology-Gynaecology-Fertility-Metabolism

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

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

Day 90 discussion

Action: For discussion

Gastroenterology-Hepatology

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

Taiga Biotechnologies, Inc; Severe combined immunodeficiency

Day 90 discussion

Action: For discussion

Immunology-Rheumatology-Transplantation

3.1.6. Ibalizumab - EMEA-002311-PIP01-17

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

Day 90 discussion

Action: For discussion

Infectious Diseases

3.1.7. Sarizotan hydrochloride - Orphan - EMEA-001808-PIP03-17

Newron Pharmaceuticals SpA; Treatment of Rett syndrome

Day 90 discussion

Action: For discussion

Neurology

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

AveXis EU Ltd; Treatment of spinal muscular atrophy Type 1

Day 90 discussion

Action: For discussion

Neurology

3.1.9. Afatinib - EMEA-001596-PIP02-17

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

Day 90 discussion

Action: For discussion

Oncology

3.1.10. Entrectinib - EMEA-002096-PIP01-16

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

Day 90 discussion

Action: For discussion

Oncology

3.1.11. Ivosidenib - Orphan - EMEA-002247-PIP03-17

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

Day 90 discussion

Action: For discussion

Oncology

3.1.12. Sulindac / Eflornithine - Orphan - EMEA-001518-PIP02-16

Cancer Prevention Pharma Ltd.; Treatment of Familial Adenomatous Polyposis

Day 90 discussion

Action: For discussion

Oncology

3.1.13. Sodium thiosulfate - EMEA-002147-PIP02-17

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

Day 90 discussion

Action: For discussion

Oncology / Oto-rhino-laryngology

3.1.14. Autologous cartilage derived cultured chondrocytes - EMEA-002217-PIP01-17

Treatment of cartilage disorders

Day 90 discussion

Action: For discussion

Other

3.1.15. Palovarotene - EMEA-001662-PIP03-17

Treatment of Multiple Osteochondromas (MO)

Day 90 discussion

Action: For discussion

Other

3.1.16. Recombinant human acid ceramidase - Orphan - EMEA-002266-PIP01-17

Enzyvant Farber Ireland Ltd; Farber disease

Day 90 discussion

Action: For discussion

Other

3.1.17. Interferon beta-1a - Orphan - EMEA-002238-PIP01-17

Faron Pharmaceuticals Ltd; Treatment of Acute Respiratory Distress Syndrome

Day 90 discussion

Action: For discussion

Pneumology - Allergology

3.1.18. EMEA-002172-PIP02-17

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

Day 90 discussion

Action: For discussion

Vaccines / Infectious Diseases

3.1.19. EMEA-002329-PIP01-18

Treatment of chronic hand eczema

Day 60 discussion

Action: For discussion

Dermatology

3.1.20. Givosiran sodium - Orphan - EMEA-002048-PIP02-18

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

Day 60 discussion

Action: For discussion

Endocrinology-Gynaecology-Fertility-Metabolism

3.1.21. DNA (synthetic adeno-associated virus vector AAV-Spark100-hFIX39-Padua) - EMEA-002362-PIP01-18

Prophylaxis of haemophilia B (hereditary factor IX deficiency)

Day 60 discussion

Action: For discussion

Haematology-Hemostaseology

3.1.22. Voxelotor - Orphan - EMEA-002356-PIP01-18

SynteractHCR Deutschland GmbH; Treatment of sickle cell disease

Day 60 discussion

Action: For discussion

Haematology-Hemostaseology

3.1.23. Ustekinumab - EMEA-000311-PIP06-18

Treatment of systemic lupus erythematosus (SLE)

Day 60 discussion

Action: For discussion

Immunology-Rheumatology-Transplantation

3.1.24. 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 60 discussion

Action: For discussion

Infectious Diseases

3.1.25. Abemaciclib - EMEA-002342-PIP01-18

Ewing's Sarcoma

Day 60 discussion

Action: For discussion

Oncology

3.1.26. Allogeneic, genetically modified T cells with inactivated T cell alpha beta receptor and CD52 protein, and expressing a CD19-specific chimeric antigen receptor and the synthetic RQR8 protein - EMEA-002348-PIP01-18

Treatment of relapse or refractory B-cell acute lymphoblastic leukemia

Day 60 discussion

Action: For discussion

Oncology

3.1.27. 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 60 discussion

Action: For discussion

Oncology

3.1.28. Pegvorhyaluronidase 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). / Pegvorhyaluronidase 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 60 discussion

Action: For discussion

Oncology

3.1.29. 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 60 discussion

Action: For discussion

Pneumology - Allergology

3.1.30. Rapastinel - EMEA-002357-PIP01-18

Major depressive disorder

Day 60 discussion

Action: For discussion

Psychiatry

3.1.31. 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 60 discussion

Action: For discussion

Vaccines

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

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

Day 30 discussion

Action: For discussion

Cardiovascular Diseases

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

ICD10:E78.2

Day 30 discussion

Action: For discussion

Cardiovascular Diseases

3.1.34. EMEA-002350-PIP01-18

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

Day 30 discussion

Action: For discussion

Dermatology

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

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

Day 30 discussion

Action: For discussion

Endocrinology-Gynaecology-Fertility-Metabolism

3.1.36. Asciminib - EMEA-002347-PIP01-18

Treatment of Philadelphia positive Chronic Myelogenous Leukemia in chronic phase

Day 30 discussion

Action: For discussion

Haematology-Hemostaseology

3.1.37. Concizumab - Orphan - EMEA-002326-PIP03-18

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

Day 30 discussion

Action: For discussion

Haematology-Hemostaseology

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

Treatment of Systemic Lupus Erythematosus (SLE)

Day 30 discussion

Action: For discussion

Immunology-Rheumatology-Transplantation

3.1.39. Fenebrutinib - EMEA-002349-PIP01-18

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

Day 30 discussion

Action: For discussion

Immunology-Rheumatology-Transplantation

3.1.40. Guselkumab - EMEA-001523-PIP03-18

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

Day 30 discussion

Action: For discussion

Immunology-Rheumatology-Transplantation

3.1.41. Rilpivirine EMEA-000317-PIP02-18

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

Day 30 discussion

Action: For discussion

Infectious Diseases

3.1.42. Suvratoxumab anti-Staphylococcus aureus alpha toxin monoclonal antibody) - EMEA-002337-PIP01-18

Prevention of nosocomial pneumonia caused by Staphylococcus aureus

Day 30 discussion

Action: For discussion

Infectious Diseases

3.1.43. Arimoclomol citrate - Orphan - EMEA-001748-PIP02-18

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

Day 30 discussion

Action: For discussion

Neurology

3.1.44. Avapritinib - Orphan - EMEA-002358-PIP02-18

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

Day 30 discussion

Action: For discussion

Oncology

3.1.45. Elotuzumab - EMEA-002377-PIP01-18

Treatment of multiple myeloma

Day 30 discussion

Action: For discussion

Oncology

3.1.46. Nadofaragene firadenovec - EMEA-002376-PIP01-18

Mesothelioma

Day 30 discussion

Action: For discussion

Oncology

3.1.47. Spartalizumab - EMEA-002351-PIP01-18

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

Day 30 discussion

Action: For discussion

Oncology

3.1.48. Tepotinib - EMEA-002345-PIP01-18

Treatment of lung malignant neoplasms

Day 30 discussion

Action: For discussion

Oncology

3.1.49. Dexamethasone / Levofloxacin - EMEA-002375-PIP01-18

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

Day 30 discussion

Action: For discussion

Ophthalmology

3.1.50. Odiparcil - Orphan - EMEA-002256-PIP01-17

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

Day 30 discussion

Action: For discussion

Other

3.1.51. Octenidine dihydrochloride - EMEA-001384-PIP02-17

Maintenance of oral hygiene / For temporary reduction of bacterial count in the oral cavity, for inhibition of plaque formation, in cases of insufficient oral hygiene capacity

Day 30 discussion

Action: For discussion

Other / Infectious Diseases

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

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

Day 30 discussion

Action: For discussion

Oto-rhino-laryngology

3.1.53. Fasinumab - EMEA-002059-PIP01-16

Chronic pain

Day 30 discussion

Action: For discussion

Pain

3.1.54. EMEA-002324-PIP01-17

Treatment of Cystic Fibrosis

Day 30 discussion

Action: For discussion

Pneumology - Allergology

3.1.55. EMEA-002191-PIP02-17

Treatment of Cystic Fibrosis

Day 30 discussion

Action: For discussion

Pneumology - Allergology

3.1.56. EMEA-002307-PIP01-17

Prevention of Ebola Virus Disease / Prevention of EVD in children aged ≥1 year

Day 30 discussion

Action: For discussion

Vaccines / Infectious Diseases

3.1.57. EMEA-002308-PIP01-17

Prevention of Ebola Virus Disease / Prevention of EVD in children aged ≥1 year

Day 30 discussion

Action: For discussion

Vaccines / Infectious Diseases

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. Macitentan - EMEA-C2-001032-PIP01-10-M02

Actelion Registration Ltd; Treatment of pulmonary arterial hypertension

Day 30 discussion

Action: For discussion

Cardiovascular Diseases

3.2.2. Trifarotene - EMEA-C-001492-PIP01-13-M01

GALDERMA R&D; Treatment of acne

Day 30 discussion

Action: For discussion

Dermatology

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

bluebird bio (Germany) GmbH; Treatment of β-thalassaemia

Day 30 discussion

Action: For discussion

Haematology-Hemostaseology

3.2.4. Upadacitinib - EMEA-C1-001741-PIP01-14-M01

AbbVie Ltd; Treatment of Chronic Idiopathic Arthritis (including rheumathoid arthritis,

psoriatic arthritis, spondyloarthritis and juvenile idiopathic arthritis)

Day 30 discussion

Action: For discussion

Immunology-Rheumatology-Transplantation

3.2.5. Fenfluramine hydrochloride - EMEA-C2-001990-PIP01-16

Zogenix International Ltd; Treatment of Dravet syndrome

Day 30 discussion

Action: For discussion

Neurology

3.2.6. Nusinersen - EMEA-C-001448-PIP01-13-M03

Biogen Idec Ltd; Treatment of spinal muscular atrophy

Day 30 discussion

Action: For discussion

Neurology

3.2.7. Siponimod fumaric acid co-crystal - EMEA-C1-000716-PIP01-09-M02

Novartis Europharm Limited; Treatment of Multiple Sclerosis

Day 30 discussion

Action: For discussion

Neurology

3.2.8. Sunitinib malate - EMEA-C-000342-PIP01-08-M07

Pfizer Limited; Treatment of gastro-intestinal stromal tumours

Day 30 discussion

Action: For discussion

Oncology

3.3. Discussions on Modification of an Agreed Paediatric Investigation Plan

3.3.1. Gadolinium,[α3,α6,α9-tris[3-[[2-hydroxy-1-(hydroxymethyl)ethyl]amino]-3-oxopropyl]-3,6,9,15-tetraazabicyclo[9.3.1]pentadeca-1(15),11,13-triene-3,6,9-triacetato(3-)-κN3,κN6,κN9,κN15,κO3,κO6,κO9] - EMEA-001949-PIP01-16-M02

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

Day 30 discussion

Action: For discussion

Diagnostic

3.3.2. Dapagliflozin - EMEA-000694-PIP01-09-M07

AstraZeneca AB; Treatment of Type 2 Diabetes

Day 30 discussion

Action: For discussion

Endocrinology-Gynaecology-Fertility-Metabolism

3.3.3. Ertugliflozin L-PGA - EMEA-001533-PIP01-13-M01

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

Day 30 discussion

Action: For discussion

Endocrinology-Gynaecology-Fertility-Metabolism

3.3.4. Exenatide - EMEA-000689-PIP01-09-M08

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

Day 30 discussion

Action: For discussion

Endocrinology-Gynaecology-Fertility-Metabolism

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

Protalix Ltd; Treatment of Fabry disease

Day 30 discussion

Action: For discussion

Endocrinology-Gynaecology-Fertility-Metabolism

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

Norgine Limited; Bowel cleansing prior to clinical procedures

Day 30 discussion

Action: For discussion

Gastroenterology-Hepatology

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

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

Day 30 discussion

Action: For discussion

Gastroenterology-Hepatology

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

Ablynx NV; Treatment of thrombotic thrombocytopenic purpura / Treatment of acquired thrombotic thrombocytopenic purpura

Day 30 discussion

Action: For discussion

Haematology-Hemostaseology

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

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

Day 30 discussion

Action: For discussion

Haematology-Hemostaseology

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

Biotest AG; Treatment of congenital fibrinogen deficiency

Day 30 discussion

Action: For discussion

Haematology-Hemostaseology

3.3.11. Belimumab - EMEA-000520-PIP02-13-M02

Glaxo Group Limited; Treatment of systemic lupus erythematosus

Day 30 discussion

Action: For discussion

Immunology-Rheumatology-Transplantation

3.3.12. Emapalumab - Orphan - EMEA-002031-PIP01-16-M02

Novimmune B.V; Treatment of Haemophagocytic Lymphohistiocytosis

Day 30 discussion

Action: For discussion

Immunology-Rheumatology-Transplantation

3.3.13. Ustekinumab - EMEA-000311-PIP03-11-M04

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

Day 30 discussion

Action: For discussion

Immunology-Rheumatology-Transplantation

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

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

Day 30 discussion

Action: For discussion

Infectious Diseases

3.3.15. Oseltamivir phosphate - EMEA-000365-PIP01-08-M10[1]

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 60 discussion

Action: For discussion

Infectious Diseases

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

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

Day 30 discussion

Action: For discussion

Infectious Diseases

3.3.17. Simeprevir - EMEA-000625-PIP01-09-M03

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

Day 30 discussion

Action: For discussion

Infectious Diseases

3.3.18. Tenofovir alafenamide / emtricitabine / cobicistat / darunavir - EMEA-001825-PIP01-15-M02

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

Day 30 discussion

Action: For discussion

Infectious Diseases

[1] Correction of the timeline for discussion

3.3.19. Tenofovir Alafenamide / Emtricitabine / Cobicistat / Elvitegravir - EMEA-001460-PIP01-13-M03

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

Day 30 discussion

Action: For discussion

Infectious Diseases

3.3.20. Tenofovir disoproxil / rilpivirine / emtricitabine - EMEA-000774-PIP01-09-M03

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

Day 30 discussion

Action: For discussion

Infectious Diseases

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

bluebird bio France; Adrenoleukodystrophy

Day 30 discussion

Action: For discussion

Neurology

3.3.22. Perampanel - EMEA-000467-PIP01-08-M10

Eisai Europe Limited; Treatment of treatment-resistant epilepsies / Adjunctive therapy in patients with other paediatric epilepsies, Adjunctive therapy in patients with refractory partial onset seizures including secondarily generalised seizures

Day 30 discussion

Action: For discussion

Neurology

3.3.23. Retigabine - EMEA-000116-PIP01-07-M09

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

partial onset seizures

Day 30 discussion

Action: For discussion

Neurology

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

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

Day 30 discussion

Action: For discussion

Oncology

3.3.25. Quizartinib - Orphan - EMEA-001821-PIP01-15-M02

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

Day 30 discussion

Action: For discussion

Oncology

3.3.26. Eliglustat - Orphan - EMEA-000461-PIP02-11-M03

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

Day 30 discussion

Action: For discussion

Other

3.3.27. Vamorolone - Orphan - EMEA-001794-PIP02-16-M01

ReveraGen BioPharma Ltd; Treatment of Duchenne muscular dystrophy

Day 30 discussion

Action: For discussion

Other

3.3.28. Dupilumab - EMEA-001501-PIP02-13-M03

sanofi-aventis recherche & développement; Asthma

Day 30 discussion

Action: For discussion

Pneumology - Allergology

3.3.29. Mepolizumab - Orphan - EMEA-000069-PIP04-13-M02

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

Day 30 discussion

Action: For discussion

Pneumology - Allergology

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

Novartis Europharm Limited; Treatment of asthma

Day 30 discussion

Action: For discussion

Pneumology - Allergology

3.3.31. Brexpiprazole - EMEA-001185-PIP01-11-M05

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

Day 30 discussion

Action: For discussion

Psychiatry

3.3.32. Cariprazine hydrochloride - EMEA-001652-PIP01-14-M02

Gedeon Richter Plc.; F20 Schizophrenia

Day 30 discussion

Action: For discussion

Psychiatry

3.3.33. Loxapine - EMEA-001115-PIP01-10-M06

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

Day 30 discussion

Action: For discussion

Psychiatry

3.3.34. Lanthanum carbonate hydrate - EMEA-000637-PIP02-10-M06

Shire Pharmaceutical Contracts Ltd; Hyperphosphataemia

Day 30 discussion

Action: For discussion

Uro-nephrology

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 21 August 2018 for Nomination of Rapporteur and Peer reviewer

Action: For adoption

4.2. Nomination of Rapporteur for requests of confirmation on the applicability of the EMA decision on class waiver.

Action: For adoption

4.3. Nominations for other activities

Action: For adoption

5. Scientific Advice Working Party (SAWP) and Paediatric Committee (PDCO) Interaction

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

6. Discussion on the applicability of class waivers

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

6.1. Discussions on the applicability of class waiver for products

6.1.1. Dupilumab – EMEA-06-2018

sanofi-aventis Recherche & Développement; All classes of medicinal products for treatment of chronic obstructive pulmonary disease (COPD) (excluding chronic lung diseases associated with long-term airflow limitation, such as asthma, bronchopulmonary dysplasia, primary cilia dyskinesia, obstructive lung disease related to graft-versus-host disease after [bone-marrow] transplantation)/Add-on maintenance treatment to reduce the risk of COPD exacerbations and improve lung function in patients with moderate-to-very severe COPD

Action: For adoption

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

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

7.1.1. Vonicog alfa - Orphan - EMEA-001164-PIP01-11-M01

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.

Proposed indication: Prophylaxis and treatment of bleeding in paediatric patients diagnosed with von Willebrand disease when desmopressin (DDAVP) treatment alone is ineffective or contraindicated

Action: For adoption

Haematology-Hemostaseology

8. Annual reports on deferrals

Note: The annual reports on deferrals to be noted by the members of the PDCO are flagged in the Annex B.

9. Organisational, regulatory and methodological matters

9.1. Mandate and organisation of the PDCO

9.2. Coordination with EMA Scientific Committees or CMDh-v

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

Action: For information

9.2.2. Guideline on the development of new medicinal products for the treatment of Ulcerative Colitis

PDCO member: Peter Szitanyi

Action: For adoption

9.2.3. Guideline on the development of new medicinal products for the treatment of Crohn's Disease

PDCO member: Peter Szitanyi

Action: For adoption

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

9.3.1. Non-clinical Working Group: D30 Products identified

PDCO member: Karen van Malderen

Action: For information

9.3.2. Formulation Working Group

PDCO member: Brian Aylward

Action: For information

9.3.3. Extrapolation Reflection Paper – status update

PDCO member: Dirk Mentzer

Action: For information

9.4. Cooperation within the EU regulatory network

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

Action: For information

9.5. Cooperation with International Regulators

None

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

None

9.7. PDCO work plan

None

9.8. Planning and reporting

None

10. Any other business

10.1.1. Training on paediatric requirements/legislations/initiatives in other regions – US and Japan

Action: For information

10.1.2. PDCO with Rome Foundation - Irritable Bowel Syndrome and Functional Constipation in children - collaborative papers

PDCO member: Johannes Taminiau

Action: For information

10.1.3. EC/EMA action plan to further improve the implementation of the Paediatric Regulation[2]

Scope: Outcomes and action plan

Action: For information

[2] Agenda item added (topic omitted in the previous version of the Agenda)

10.1.4. Joint CHMP & PDCO Strategic Review & Learning Meeting Vienna 26-28 September 2018

PDCO member: Karl-Heinz Huemer

10.1.5. EMA Workshop on development of antibacterial medicinal products for paediatric patients

PDCO member: Irja Lutsa

Scope: Update on the preliminary program and PDCO topics

Action: For information

11. Breakout sessions

11.1.1. Paediatric oncology

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

11.1.2. Neonatology

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

11.1.3. Inventory

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/