24 May 2016
EMA/PDCO/316468/2016
Procedure Management and Committees Support Division

Paediatric Committee (PDCO)

Draft agenda for the meeting on 25-27 May 2016

Chair: Dirk Mentzer – Vice-Chair: Koenraad Norga

25 May 2016, 08:30- 19:00, room 3E

26 May 2016, 08:30- 19:00, room 3E

27 May 2016, 08:30- 13:00, room 3E

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

Table of contents

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 on 25-27 May 2016. See May 2016 PDCO minutes (to be published post June 2016 PDCO meeting).

1.2. Adoption of agenda

PDCO agenda for 25-27 May 2016.

1.3. Adoption of the minutes

PDCO minutes for 27-29 April 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. Eleclazine - EMEA-001697-PIP01-14

Treatment of congenital long QT syndromes / Indicated for the treatment of long QT syndrome type 2 (LQT2), Indicated for the treatment of long QT syndrome type 3 (LQT3)

Day 120 opinion

Action: For adoption

Cardiovascular Diseases

2.1.2. Eleclazine - EMEA-001697-PIP02-14

Treatment of hypertrophic cardiomyopathy / Indicated for the treatment of symptomatic hypertrophic cardiomyopathy (HCM)

Day 120 opinion

Action: For adoption

Cardiovascular Diseases

2.1.3. Metreleptin - Orphan - EMEA-001701-PIP01-14

Aegerion Pharmaceuticals Ltd; Treatment of lipodystrophy

Day 120 opinion

Action: For adoption

Endocrinology-Gynaecology-Fertility-Metabolism

2.1.4. Humanized monoclonal modified immunoglobulin G4 (IgG4) antibody with bispecific structure targeting factors IX, IXa, X and Xa - Orphan - EMEA-001839-PIP01-15

Roche Registration Limited; Treatment of Hereditary FVIII Deficiency / indicated for the routine prophylaxis to reduce the frequency of or prevent bleeding episodes in paediatric patients with hemophilia A with FVIII inhibitors

Day 120 opinion

Action: For adoption

Haematology-Hemostaseology

2.1.5. Cadazolid - EMEA-001108-PIP02-15

Enterocolitis due to Clostridium difficile / Treatment of Clostridium difficile-associated diarrhea (CDAD)

Day 120 opinion

Action: For adoption

Infectious Diseases

2.1.6. cytarabine 100 mg (liposome combination) daunorubicin HCl 44mg (liposome combination) - Orphan - EMEA-001858-PIP01-15

Celator (UK) Ltd; Acute myeloid leukemia / treatment of acute myeloid leukemia

Day 120 opinion

Action: For adoption; Oral Explanation Meeting to be held on Thursday 26 May 2016, 14:00-15:00

Oncology

2.1.7. Glycopyrronium bromide (dose expressed as free base) / Mometasone furoate / Indacaterol acetate (dose expressed as free base) - EMEA-001812-PIP01-15

Treatment of asthma

Day 120 opinion

Action: For adoption

Pneumology – Allergology

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. cinacalcet - EMEA-C-000078-PIP01-07-M07

Amgen Europe B.V.; treatment of secondary hyperparathyroidism in patients with end-stage renal disease

Day 60 opinion

Action: For adoption

Uro-nephrology

2.2.2. Diphtheria toxoid-2IU / Tetanus toxoid-20IU / Bordetella pertussis antigen : Pertussis toxoid-8µg Filamentous Haemagglutinin-8µg Pertactin-2.5µg / Inactivated poliovirus: type 1 (Mahoney strain)-40D Inactivated poliovirus: type 2 (MEF-1 strain)-8D Inactivated poliovirus: type 3 (Saukett strain)-32D - EMEA-C-000500-PIP01-08-M03 – early adoption of opinion

GlaxoSmithKline Biologicals S.A; Prevention of infectious diseases caused by Corynebacterium diphtheriae / Clostridium tetani / Bordetella pertussis / Poliovirus types 1, 2 and 3

Day 30 opinion

Action: For adoption

Vaccines

2.2.3. Rufinamide- EMEA-C3-000709-PIP01-09-M05

Eisai Limited; Treatment of Lennox Gastaut Syndrome

Action: adopted via written procedure on 23 May 2016

Neurology

2.2.4. Levamisole Hydrochloride - EMEA-C-001885-PIP01-15 – early adoption of opinion

ACE Pharmaceuticals BV; treatment of glomerulonephritis and nephrotic syndrome

Day 3 opinion

Action: For adoption

Uro-nephrology

2.2.5. Tiotropium bromide (monohydrate) - EMEA-C-000035-PIP02-09-M02 – early adoption of opinion

Boehringer Ingelheim International GmbH; Treatment of asthma

Day 30 opinion

Action: For adoption

Pneumology - Allergology

2.3. Opinions on Modification of an Agreed Paediatric Investigation Plan

2.3.1. Landiolol hydrochloride - EMEA-001150-PIP02-13-M01

AOP Orphan Pharmaceuticals AG; Treatment of supraventricular arrhythmias / Treatment of sinus tachycardia or supraventricular tachyarrhythmias, including junctional ectopic tachycardia (JET), atrial flutter (AF), atrial fibrillation (AFL), focal atrial tachycardia (FAT), atrioventricular re-entrant tachycardia (AVRT), and atrioventricular nodal re-entrant tachycardia (AVNRT), peri-operatively (during an induction phase, intra-operatively, and during the weaning phase), or when in the phycisian's judgement control of the heart rate is required.

Day 60 opinion

Action: For adoption

Cardiovascular Diseases

2.3.2. rCFP-10 (recombinant 10 kD culture filtrate protein) / rdESAT-6 (recombinant dimer of 6 kD early secretory antigenic target) - EMEA-001156-PIP01-11-M07

Statens Serum Institut; Diagnosis of tuberculosis / To diagnose individuals suspected to be infected with Mycobacterium tuberculosis from 28 days of age

Day 60 opinion

Action: For adoption

Diagnostic

2.3.3. corifollitropin alfa - EMEA-000306-PIP01-08-M03

Merck Sharp & Dohme Limited; Inability to achieve pregnancy, female / hypogonadotropic hypogonadism

Day 60 opinion

Action: For adoption

Endocrinology-Gynaecology-Fertility-Metabolism

2.3.4. dulaglutide - EMEA-000783-PIP01-09-M04

Eli Lilly & Company; Type 2 diabetes mellitus

Day 60 opinion

Action: For adoption

Endocrinology-Gynaecology-Fertility-Metabolism

2.3.5. Liraglutide - EMEA-000128-PIP01-07-M07

Novo Nordisk A/S; E11 Non-insulin-dependent diabetes mellitus / Treatment of type 2 diabetes mellitus

Day 60 opinion

Action: For adoption

Endocrinology-Gynaecology-Fertility-Metabolism

2.3.6. caplacizumab (anti-von Willebrand Factor Nanobody) - Orphan - EMEA-001157-PIP01-11-M01

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

Day 60 opinion

Action: For adoption

Haematology-Hemostaseology

2.3.7. Deferasirox - Orphan - EMEA-001103-PIP01-10-M03

Novartis Europharm Limited; Treatment of chronic overload requiring chelation therapy / Treatment of chronic iron overload due to blood transfusions when deferoxamine therapy is contraindicated or inadequate in patients with others anemias, Treatment of chronic transfusional iron overload in patients with beta thalassemia major, Treatment of chronic iron overload in patients with non-transfusion-dependent thalassemia

Day 60 opinion

Action: For adoption

Haematology-Hemostaseology

2.3.8. Eltrombopag - EMEA-000170-PIP03-13-M01

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 60 opinion

Action: For adoption

Haematology-Hemostaseology

2.3.9. baricitinib - EMEA-001220-PIP01-11-M01

Eli Lilly & Company Limited; Treatment of chronic idiopathic arthritis (including rheumatoid arthritis, ankylosing spondylarthritis, psoriatic arthritis and juvenile idiopathic arthritis) / Treatment of juvenile idiopathic arthritis, Treatment of JIA-associated uveitis

Day 60 opinion

Action: For adoption

Immunology-Rheumatology-Transplantation

2.3.10. belimumab - EMEA-000520-PIP01-08-M05

Glaxo Group Limited; Systemic lupus erythematosus / Treatment of systemic lupus erythematosus

Day 60 opinion

Action: For adoption

Immunology-Rheumatology-Transplantation

2.3.11. Eculizumab - Orphan - EMEA-000876-PIP05-15-M01

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

Day 60 opinion

Action: For adoption

Immunology-Rheumatology-Transplantation

2.3.12. letermovir - Orphan - EMEA-001631-PIP01-14-M01

Merck Sharp & Dohme (Europe), Inc.; Prevention of cytomegalovirus infection / Prevention of CMV viremia and/or disease in at-risk patients having undergone an allogeneic HSCT or SOT

Day 60 opinion

Action: For adoption

Infectious Diseases

2.3.13. EMEA-001411-PIP01-12-M03

Gilead Sciences International Ltd; Chronic Viral Hepatitis C infection / Chronic Viral Hepatitis

C infection

Day 60 opinion

Action: For adoption

Infectious Diseases

2.3.14. Brivaracetam - Orphan - EMEA-000332-PIP01-08-M10

UCB Pharma SA; Treatment of epilepsy with partial onset seizures, Treatment of neonatal seizures / Treatment of neonatal seizures with adjunctive administration of brivaracetam, Treatment of paediatric patients with partial onset seizures

Day 60 opinion

Action: For adoption

Neurology

2.3.15. Decitabine - Orphan - EMEA-000555-PIP01-09-M05

Janssen-Cilag International NV; Acute Myeloid Leukaemia / Treatment of paediatric patients with acute myeloid leukaemia who have high-risk cytogenetics, or are refractory to, or have a relapse after first line treatment

Day 60 opinion

Action: For adoption

Oncology

2.3.16. Regorafenib - EMEA-001178-PIP01-11-M02

Bayer Pharma; Treatment of all conditions contained in the category of malignant neoplasms (except haematopoietic and lymphoid tissue) / Treatment of paediatric patients with a solid malignant tumour(s) integrated with anti-cancer therapy

Day 60 opinion

Action: For adoption

Oncology

2.3.17. Methoxyflurane - EMEA-000334-PIP01-08-M04

Medical Developments UK Ltd; treatment of acute pain

Day 60 opinion

Action: For adoption; Oral Explanation Meeting to be held on Wednesday 25 May 2016; 11:00 - 12:00

Pain

2.3.18. 1-(2,2-difluoro-1,3-benzodioxol-5-yl)-N-{1-[(2R)-2,3-dihydroxypropyl]-6-fluoro-2-(1-hydroxy-2-methylpropan-2-yl)-1H-indol-5-yl}cyclopropanecarboxamide / Ivacaftor - Orphan - EMEA-001640-PIP01-14-M01

Vertex Pharmceuticals (Europe) Ltd; Cystic Fibrosis / Treatment of Cystic Fibrosis

Day 60 opinion

Action: For adoption

Pneumology - Allergology

2.3.19. AGOMELATINE - EMEA-001181-PIP01-11-M03

Les Laboratoires Servier; Major Depressive Episodes / Major Depressive Episodes

Day 60 opinion

Action: For adoption

Psychiatry

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. betrixaban - EMEA-001834-PIP01-15

Prevention of venous thromboembolism / adults and children

Day 90 discussion

Action: For discussion

Cardiovascular Diseases

3.1.2. Semaglutide - EMEA-001441-PIP02-15

Treatment of Type 2 Diabetes Mellitus / Treatment of Type 2 Diabetes Mellitus

Day 90 discussion

Action: For discussion

Endocrinology-Gynaecology-Fertility-Metabolism

3.1.3. EMEA-001843-PIP01-15

Chronic Idiopathic Arthritis / Treatment of Juvenile Idiopathic Arthritis

Day 90 discussion

Action: For discussion

Immunology-Rheumatology-Transplantation

3.1.4. Eculizumab - Orphan - EMEA-000876-PIP06-15

Alexion Europe SAS; Prevention of graft rejection following solid organ transplantation / Prevention of acute antibody-mediated rejection in sensitized recipients after kidney transplantation

Day 90 discussion

Action: For discussion

Immunology-Rheumatology-Transplantation

3.1.5. EMEA-001776-PIP01-15

Treatment of Active Psoriatic Arthritis, Treatment of Crohn's disease, Treatment of plaque psoriasis, Treatment of Ankylosing Spondylitis, Treatment of Asthma, Treatment of moderate to severe plaque psoriasis in children and adolescents from the age of 6 years, who are candidates for systemic therapies

Day 90 discussion

Action: For discussion

Immunology-Rheumatology-Transplantation / Dermatology / Pneumology - Allergology / Gastroenterology-Hepatology

3.1.6. EMEA-001838-PIP01-15

Treatment of respiratory tract disease caused by human respiratory syncytial virus (RSV) / Treatment of respiratory tract disease caused by human RSV

Day 90 discussion

Action: For discussion

Infectious Diseases

3.1.7. Cabotegravir - EMEA-001418-PIP02-15

Prevention of human immunodeficiency virus (HIV-1) infection / Cabotegravir is to be indicated in combination with safer sex practices for PrEP to reduce the risk of HIV-1 acquisition in sexually active adolescents at high risk, from 12 to < 18 years of age

Day 90 discussion

Action: For discussion

Infectious Diseases

3.1.8. Peramivir - EMEA-001856-PIP01-15

Treatment of influenza / Treatment of influenza

Day 90 discussion

Action: For discussion

Infectious Diseases

3.1.9. Quizartinib - Orphan - EMEA-001821-PIP01-15

Daiichi Sankyo Development Ltd; Acute myeloid leukaemia / For the treatment of paediatric patients aged from 1 month to less than 18 years of age with newly diagnosed FLT3-ITD(+) AML, For the treatment of paediatric patients aged from 1 month to less than 18 years of age with relapsed or refractory FLT3-ITD(+) AML after failure of front line intensive chemotherapy regimen, in combination with standard chemotherapy

Day 90 discussion

Action: For discussion

Oncology

3.1.10. andexanet alfa - EMEA-001902-PIP01-15

Prevention of factor Xa inhibitor associated haemorrhage, Treatment of factor Xa inhibitor associated haemorrhage / (as above), For the reversal of anticoagulation due to direct and indirect factor Xa inhibitors in patients experiencing an acute major bleeding event or requiring urgent surgery.

Day 90 discussion

Action: For discussion

Other

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

GlaxoSmithKline Trading Services Limited; Metachromatic leukodystrophy (MLD) / For the

treatment of metachromatic leukodystrophy (MLD)

Day 90 discussion

Action: For discussion

Other

3.1.12. 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 60 discussion

Action: For discussion

Gastroenterology-Hepatology

3.1.13. alvimopan - EMEA-001922-PIP01-15

Postoperative ileus

Day 60 discussion

Action: For discussion

Gastroenterology-Hepatology

3.1.14. Susoctocog alfa - EMEA-000753-PIP02-16

Congenital haemophilia A with antibodies (inhibitors) to human factor VIII / Peri-operative management in patients with congenital haemophilia A with antibodies (inhibitors) to human FVIII, On-demand treatment and control of bleeding episodes in patients with congenital haemophilia A with antibodies (inhibitors) to human FVIII

Day 60 discussion

Action: For discussion

Haematology-Hemostaseology

3.1.15. Fc- and CDR-modified humanized monoclonal antibody against C5 - EMEA-001943-PIP01-16

Atypical Haemolytic Uremic Syndrome / Treatment of atypical Haemolytic Uremic Syndrome

Day 60 discussion

Action: For discussion

Uro-nephrology / Haematology-Hemostaseology

3.1.16. Allogeneic Human Adult Mesodermal Immunomodulatory Progenitor Cells - EMEA-001955-PIP01-16

Heart Failure

Day 30 discussion

Action: For discussion

Cardiovascular Diseases

3.1.17. Rosuvastatin / Amlodipine - EMEA-001935-PIP01-16

Treatment of angina and dyslipidaemia, Treatment of concomitant hypertension and dyslipidemia, Treatment of essential hypertension in patients who are estimated to have a high risk for a first cardiovascular event

Day 30 discussion

Action: For discussion

Cardiovascular Diseases

3.1.18. 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 30 discussion

Action: For discussion

Endocrinology-Gynaecology-Fertility-Metabolism

3.1.19. EMEA-001929-PIP01-16

Crohn's disease, Ulcerative colitis / Treatment of children 4 to 17 years of age with moderately to severely active Crohn's disease who have had an inadequate response with, lost response to, or were intolerant to treatment with a tumour necrosis factor-alpha inhibitor; or immunomodulator, or had an inadequate response with, were intolerant to, or demonstrated dependence on corticosteroids., Treatment of children 4 to 17 years of age with moderately to severely active ulcerative colitis who have had an inadequate response with, lost response to, or were intolerant to treatment with a tumour necrosis factor-alpha inhibitor; or immunomodulator, or had an inadequate response with, were intolerant to, or demonstrated dependence on corticosteroids.

Day 30 discussion

Action: For discussion

Gastroenterology-Hepatology

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

Beta-thalassemia major and intermedia / Treatment of Beta thalassemia major and intermedia

Day 30 discussion

Action: For discussion

Haematology-Hemostaseology

3.1.21. EMEA-001944-PIP01-16

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

Day 30 discussion

Action: For discussion

Haematology-Hemostaseology

3.1.22. Lusutrombopag - EMEA-001905-PIP01-15

Treatment of thrombocytopenia

Day 30 discussion

Action: For discussion

Haematology-Hemostaseology

3.1.23. EMEA-001923-PIP01-15

Chronic idiopathic arthritis, including rheumatoid arthritis, axial spondyloarthritis, psoriatic arthritis and juvenile idiopathic arthritis (pJIA indication), Chronic idiopathic arthritis, including rheumatoid arthritis, axial spondyloarthritis, psoriatic arthritis and juvenile idiopathic arthritis (sJIA indication) / Treatment of systemic Juvenile Idiopathic Arthritis (sJIA), Treatment of polyarticular-course Juvenile Idiopathic Arthritis (pJIA).

Day 30 discussion

Action: For discussion

Immunology-Rheumatology-Transplantation

3.1.24. Dolutegravir (DTG) / Lamivudine (3TC) - EMEA-001940-PIP01-16

Treatment of human immunodeficiency virus (HIV-1) infection / Treatment of human immunodeficiency virus (HIV-1) infection

Day 30 discussion

Action: For discussion

Infectious Diseases

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

Migraine headaches / Prophylaxis of migraine

Day 30 discussion

Action: For discussion

Neurology

3.1.26. Humanized monoclonal calcitonin gene-related peptide neutralizing antibody - EMEA-001860-PIP04-16

Prophylactic treatment of cluster headache

Day 30 discussion

Action: For discussion

Neurology

3.1.27. Lenalidomide - Orphan - EMEA-000371-PIP03-15

Celgene Europe Limited; Marginal zone lymphoma, Multiple Myeloma, Follicular Lymphoma, Diffuse Large B-cell Lymphoma, Myelodysplastic syndrome, Mantle Cell Lymphoma

Day 30 discussion

Action: For discussion

Oncology

3.1.28. Pembrolizumab - EMEA-001474-PIP02-16

Treatment of all conditions included in the category of malignant neoplasms (except nervous system, haematopoietic and lymphoid tissue)., Treatment of Hodgkin Lymphoma / Treatment of relapsed or refractory classical Hodgkin Lymphoma in children from 5 years to less than 18 years of age., Treatment of advanced, untreated or previously treated, malignant melanoma in children from 12 year old to less than 18 years of age. Treatment as monotherapy of a PD-L1 positive paediatric malignant solid tumor in children from 6 months to less than 18 years of age

Day 30 discussion

Action: For discussion

Oncology

3.1.29. Recombinant human monoclonal IgG1 antibody directed against Programmed Death Ligand-1 (anti-PD-L1) - Orphan - EMEA-001849-PIP02-15

Merck KGaA; The treatment of solid malignant neoplasms

Day 30 discussion

Action: For discussion

Oncology

3.1.30. Dexamethasone / Povidone-Iodine - EMEA-001936-PIP01-16

Treatment of Infectious conjunctivitis (adenoviral and bacterial)

Day 30 discussion

Action: For discussion

Ophthalmology

3.1.31. EMEA-001947-PIP01-16

Grass pollen-induced allergic rhinitis/rhinoconjunctivitis / Treatment of grass pollen-induced allergic rhinitis, with or without conjunctivitis (AR/C)

Day 30 discussion

Action: For discussion

Pneumology - Allergology / Oto-rhino-laryngology

3.1.32. Lisdexamfetamine dimesylate - EMEA-000553-PIP02-16

Binge Eating Disorder / Binge Eating Disorder in Adults

Day 30 discussion

Action: For discussion

Psychiatry

3.1.33. Recombinant E. coli serotype O25B antigen polysaccharide (EcoO25B) – EPA (E) conjugate / Recombinant E. coli serotype O6A antigen polysaccharide (EcoO6A) – EPA (E) conjugate / Recombinant E. coli serotype O2 antigen polysaccharide (EcoO2) – EPA (E) conjugate / Recombinant E. coli serotype O1A antigen polysaccharide (EcoO1A) – EPA (E) conjugate - EMEA-001937-PIP01-16

Prevention of Escherichia infections / Full waiver for paediatric use is being requested

Day 30 discussion

Action: For discussion

Vaccines

3.1.34. Ramipril/Amlodipine/Hydrochlorothiazide - EMEA-001942-PIP01-16

Treatment of hypertension

Day 30 discussion

Action: For discussion

Cardiovascular 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. Recombinant human alpha-mannosidase; Lamazym; INN: velmanase alfa; - EMEA-C1-001056-PIP02-12

Chiesi Farmaceutici S.p.A.; Treatment of alpha-Mannosidosis

Day 30 discussion

Action: For discussion

Endocrinology-Gynaecology-Fertility-Metabolism

3.2.2. Human Fibrinogen - EMEA-C1-001208-PIP01-11-M02

Octapharma Pharamzeutika Produktionsges.m.b.H; Treatment of congenital fibrinogen deficiency

Day 30 discussion

Action: For discussion

Haematology-Hemostaseology

3.2.3. Recombinant Human TriPeptidyl Peptidase 1 (rhTPP1) - EMEA-C-001362-PIP01-12-M02

BioMarin International Limited; Treatment of Neuronal Ceroid Lipofuscinosis Type 2 (CLN2)

Day 30 discussion

Action: For discussion

Neurology

3.3. Discussions on Modification of an Agreed Paediatric Investigation Plan

3.3.1. Dobutamine - EMEA-001262-PIP01-12-M01

Proveca Limited; Circulatory impairment / haemodynamic insufficiency

Day 30 discussion

Action: For discussion

Cardiovascular Diseases

3.3.2. Recombinant human beta-glucuronidase (rhGUS, UX003) - Orphan - EMEA-001540-PIP01-13-M01

Ultragenyx UK Limited; ICD-10: E76.2, Mucopolysaccharidosis type 7 (MPS 7) / Treatment of Mucopolysaccharidosis 7 (MPS 7)

Day 30 discussion

Action: For discussion

Endocrinology-Gynaecology-Fertility-Metabolism

3.3.3. elobixibat - EMEA-001484-PIP01-13-M01

Elobix AB; Constipation

Day 30 discussion

Action: For discussion

Gastroenterology-Hepatology

3.3.4. turoctocog alfa - EMEA-000428-PIP01-08-M03

Novo Nordisk A/S; Hereditary Factor VIII Deficiency / Treatment and prophylaxis of bleeding in patients with Haemophilia A (congenital Factor VIII deficiency)

Day 30 discussion

Action: For discussion

Haematology-Hemostaseology

3.3.5. Adalimumab - EMEA-000366-PIP05-12-M02

AbbVie Limited; Non-infectious uveitis

Day 30 discussion

Action: For discussion

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

3.3.6. (3-((4-Benzoyl-1-piperazinyl)(oxo)acetyl)-4-methoxy-7-(3-methyl-1H-1,2,4-triazol-1-yl)-1H-pyrrolo[2,3-c]pyridin-1-yl)methyl dihydrogen phosphate, 2-amino-2-(hydroxymethyl)-1,3-propanediol (1:1) - EMEA-001687-PIP01-14-M01

Bristol-Myers Squibb International Corporation; Treatment of human immunodeficiency virus [HIV-1] infection / Treatment of multi-drug resistant HIV-1 infection as part of a combination therapy in paediatric patients aged 2 years to <18 years, who have no more than 2 remaining available fully active antiretroviral therapies

Day 30 discussion

Action: For discussion

Infectious Diseases

3.3.7. Anidulafungin - EMEA-000469-PIP01-08-M06

Pfizer Limited; Treatment of invasive candidiasis

Day 30 discussion

Action: For discussion

Infectious Diseases

3.3.8. rilpivirine (as hydrochloride) - EMEA-000317-PIP01-08-M09

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

3.3.9. Tenofovir alafenamide (as fumarate) - EMEA-001584-PIP01-13-M01

Gilead Sciences International Ltd.; Treatment of chronic hepatitis B / Indicated for the treatment of chronic hepatitis B infection in paediatric patients aged 2 years and above.

Day 30 discussion

Action: For discussion

Infectious Diseases

3.3.10. Brentuximab vedotin - Orphan - EMEA-000980-PIP01-10-M04

Takeda Pharma A/S; Treatment of Hodgkin lymphoma, Treatment of anaplastic large cell lymphoma / Treatment of paediatric patients with newly diagnosed, relapsed or refractory Hodgkin lymphoma (from 5 years of age), Treatment of paediatric patients with first and

subsequent relapse or refractory systemic anaplastic large cell lymphoma (from 2 years of age)

Day 30 discussion

Action: For discussion

Oncology

3.3.11. Dinutuximab - Orphan - EMEA-001285-PIP01-12-M02

United Therapeutics Europe Limited; Neuroblastoma / Treatment of patients with high-risk neuroblastoma following myeloablative therapy and autologous stem cell rescue in combination with GM-CSF, IL-2, and isotretinoin.

Day 30 discussion

Action: For discussion

Oncology

3.3.12. vemurafenib (propane-1-sulfonic acid {3-[5-(4-chlorophenyl)-1H-pyrrolo[2,3-b]pyridine-3-carbonyl]-2,4-difluorophenyl}-amide - EMEA-000978-PIP01-10-M01

Roche Registration Limited; Treatment of melanoma

Day 30 discussion

Action: For discussion

Oncology

3.3.13. ivacaftor / lumacaftor - EMEA-001582-PIP01-13-M04

Vertex Pharmaceuticals (Europe) Limited; cystic fibrosis / Treatment of cystic fibrosis

Day 30 discussion

Action: For discussion

Other

3.3.14. Benralizumab - EMEA-001214-PIP01-11-M05

AstraZeneca AB; Asthma / Treatment of asthma

Day 30 discussion

Action: For discussion

Pneumology - Allergology

3.3.15. budesonide - EMEA-001087-PIP02-12-M02

Vectura Limited; treatment of asthma

Day 30 discussion

Action: For discussion

Pneumology - Allergology

3.3.16. lurasidone hydrochloride - EMEA-001230-PIP01-11-M02

Sunovion Pharmaceuticals Ltd.; schizophrenia / schizophrenia

Day 30 discussion

Action: For discussion

Psychiatry

3.3.17. Pneumococcal polysaccharide serotype 23F conjugated to protein D (derived from non-typeable Haemophilus influenzae) carrier protein / Pneumococcal polysaccharide serotype 19F conjugated to diphtheria toxoid / Pneumococcal polysaccharide serotype 18C conjugated to tetanus toxoid / Pneumococcal polysaccharide serotype 14 conjugated to protein D (derived from non-typeable Haemophilus influenzae) carrier protein / Pneumococcal polysaccharide serotype 9V conjugated to protein D (derived from non-typeable Haemophilus influenzae) carrier protein / Pneumococcal polysaccharide serotype 7F conjugated to protein D (derived from non-typeable Haemophilus influenzae) carrier protein / Pneumococcal polysaccharide serotype 6B conjugated to protein D (derived from non-typeable Haemophilus influenzae) carrier protein / Pneumococcal polysaccharide serotype 5 conjugated to protein D (derived from non-typeable Haemophilus influenzae) carrier protein / Pneumococcal polysaccharide serotype 1 conjugated to protein D (derived from non-typeable Haemophilus influenzae) carrier protein / Pneumococcal polysaccharide serotype 4 conjugated to protein D (derived from non-typeable Haemophilus influenzae) carrier protein - EMEA-000673-PIP01-09-M09

GlaxoSmithKline Biologicals S.A.; Disease caused by Streptococcus pneumoniae, Acute Otitis Media caused by Non-typeable Haemophilus influenzae / Disease caused by Streptococcus pneumoniae, Acute Otitis Media caused by Non-typeable Haemophilus influenzae

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 19 July 2016 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

4.3.1. Call for expression of interest to become PDCO representative in Enpr-EMA Coordinating Group

Scope: Replacement of Christoph Male

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. Durvalumab , Tremelimumab - EMEA-15-2016

Treatment of ureter and bladder carcinoma; Durvalumab monotherapy for the treatment of patients with inoperable or metastatic urothelial cancer (UC) who express PD-L1 and have progressed during or after 1 prior line of therapy; Durvalumab monotherapy and durvalumab in combination with tremelimumab for first line treatment of patients with unresectable stage IV urothelial bladder cancer

Action: For adoption

6.1.2. NGR-human Tumor Necrosis Factor alpha (NGR-hTNF) - EMEA-16-2016

Treatment of mesothelioma/ Treatment of adult patients with advanced malignant pleural mesothelioma who have progressed within six months after a pemetrexed-based first-line therapy

Action: For adoption

6.1.3. Pembrolizumab - EMEA-17-2016

Treatment of multiple myeloma; Treatment of patients with refractory or relapsed and refractory multiple myeloma; Treatment of patients with newly diagnosed and naïve multiple myeloma

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

None

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. Nomination of PDCO representative at the Working Party with Healthcare Professionals' Organisations (HCPWP) and Patients' and Consumers' Working Party (PCWP)

PDCO Chair: Dirk Mentzer

Action: For adoption

9.2.3. Reflection paper on collecting and reporting information on off-label use in pharmacovigilance

Action: For information

9.2.4. GVP module VI on Management and reporting of adverse reactions to medicinal products - revision 2

Action: For information

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

9.4. Cooperation within the EU regulatory network

9.4.1. Feedback on EU Network Training Centre (EU NTC) Paediatric Curriculum

Action: For discussion

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

9.5.2. Report from the workshop 'Successes and Challenges of Performing Long-Term Paediatric Safety Studies' organised by the Food and Drug Administration (FDA) on 13-14 April 2016

PDCO Chair: Dirk Mentzer

Action: For information

9.5.3. Report from the 'EMA public workshop on extrapolation of efficacy and safety in medicine development' held on 17 May 2016

Action: For information

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

None

9.7. PDCO work plan

None

9.8. Planning and reporting

None

9.9. PDCO ORGAM

None

10. Any other business

10.1.1. Templates for the summaries of the PDCO opinions

Action: For discussion

10.1.2. Presentation of Business Pipeline activity

Action: For information

10.1.3. Simplification of members' access to EMA decisions including PDCO opinions and summary reports

Action: For information

11. Breakout sessions

11.1.1. Paediatric oncology

Action: For discussion on Thursday, 18:00 - 18:30, room 3M

11.1.2. Neonatology

Action: For discussion on Thursday, 18:00 - 18:30, room 3H

11.1.3. Inventory

Action: For discussion on Thursday, 18:00 - 18:30, 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/