16 August 2016
EMA/PDCO/517532/2016
Procedure Management and Committees Support Division

Paediatric Committee (PDCO)

Draft agenda for the meeting on 17-19 August 2016

Chair: Dirk Mentzer – Vice-Chair: Koenraad Norga

17 August 2016, 08:30- 19:00, room 3A

18 August 2016, 08:30- 19:00, room 3A

19 August 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 17-19 August 2016. See September 2016 PDCO minutes (to be published post September 2016 PDCO meeting).

1.2. Adoption of agenda

PDCO agenda for 17-19 August 2016.

1.3. Adoption of the minutes

PDCO minutes for 20-22 July 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. Bivalent anti-human myostatin adnectin recombinant human IgG1-Fc fusion protein - EMEA-001793-PIP01-15

Treatment of Duchenne Muscular Dystrophy / Treatment of Duchenne Muscular Dystrophy in patients aged ≥2 years to <18 years

Day 120 opinion

Action: For adoption

Neurology

2.1.2. GIVINOSTAT - Orphan - EMEA-000551-PIP02-14

Italfarmaco S.p.A.; Duchenne Muscular Dystrophy / Improvement of symptoms and improvement of disability in DMD affected patients

Day 120 opinion

Action: For adoption

Neurology

2.1.3. copanlisib - EMEA-001757-PIP02-15

Treatment of all conditions included in the category of malignant neoplasms (except hematopoietic and lymphoid tissue)., Treatment of mature B-cell neoplasms / , Treatment of children with neuroblastoma, Ewing's sarcoma, osteosarcoma or rhabdomyosarcoma who failed one or more prior lines of therapy.

Day 120 opinion

Action: For adoption

Oncology

2.1.4. Guadecitabine / Guadecitabine - EMEA-001730-PIP02-15

Treatment of acute myeloid leukemia / Adults with Previously Untreated Acute Myeloid Leukemia (AML) Who Are Not Considered Candidates or unfit for Intensive Remission Induction Chemotherapy, Treatment of pediatric subjects age 3 months or older to less than 18 years with relapsed refractory AML after failure of intensive remission induction chemotherapy

Day 120 opinion

Action: For adoption

Oncology

2.1.5. Orphan - EMEA-001794-PIP01-15

ReveraGen BioPharma Ltd; Treatment of duchenne muscular dystrophy / Treatment of duchenne muscular dystrophy

Day 120 opinion

Action: For adoption

Other

2.1.6. Recombinant, CHO cell expressed, fully human IgG1, kappa light chain, monoclonal antibody - Orphan - EMEA-001864-PIP01-15

Dyax Corp.; Hereditary angioedema / Treatment of hereditary angioedema

Day 120 opinion

Action: For adoption

Other

2.1.7. Ragweed pollen extract (Ambrosia artemisiifolia) - EMEA-001881-PIP01-15

treatment of allergic rhinitis and/or conjunctivitis / treatment of ragweed pollen allergic rhinitis and/or conjunctivitis

Day 120 opinion

Action: For adoption

Pneumology - Allergology

2.1.8. fluoromisonidazolum (18F) - EMEA-001977-PIP02-16

Imaging of hypoxic tissue in Non-small Cell Lung Cancer (NSCLC) for diagnostic purposes, Imaging of hypoxic tissue in Renal Cell Carcinoma (RCC) for diagnostic purposes, Imaging of hypoxic tissue in Gliomas for diagnostic purposes, Imaging of hypoxic tissue in Head and Neck Squamous Cell Carcinoma (HNSCC) for diagnostic purposes

Day 60 opinion

Action: For adoption

Diagnostic / Oncology

2.1.9. Recombinant Respiratory Syncytial Virus Vaccine with adjuvant - EMEA-001966-PIP01-16

Prevention of RSV disease

Day 60 opinion

Action: For adoption

Infectious Diseases

2.1.10. lifitegrast - EMEA-001979-PIP01-16

Treatment of dry eye disease

Day 60 opinion

Action: For adoption

Ophthalmology

2.1.11. paracetamol / ibuprofen - EMEA-002002-PIP01-16

Treatment of pain

Day 60 opinion

Action: For adoption

Pain

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. pazopanib - EMEA-C4-000601-PIP01-09-M03

Novartis Europharm Limited; Ewing sarcoma family of tumours

Day 30 opinion

Action: For adoption

Oncology

2.2.2. Pitavastatin calcium - EMEA-C-000054-PIP01-07-M04

Kowa Pharmaceutical Europe Co. Ltd.; Treatment of disorders of lipoprotein metabolism and other lipidaemias

Day 60 opinion

Action: For adoption

Endocrinology-Gynaecology-Fertility-Metabolism

2.2.3. Pitavastatin calcium - EMEA-C-000300-PIP01-08-M04

Kowa Pharmaceutical Europe Co. Ltd.; Treatment of disorders of lipoprotein metabolism and other lipidaemias

Day 60 opinion

Action: For adoption

Endocrinology-Gynaecology-Fertility-Metabolism

2.2.4. icatibant - EMEA-C-000408-PIP01-08-M05

Shire Orphan Therapies GmbH; Treatment of hereditary angioedema (HAE)

Day 60 opinion

Action: For adoption

Other

2.2.5. Solithromycin – EMEA-C2-001581-PIP01-13-M03

Triskel EU Services, Ltd; Treatment of bacterial pneumonia/Treatment of tularaemia/ Treatment of anthrax

Day 1 opinion

Action: For information; compliance adopted via written procedure on 9 August 2016

2.3. Opinions on Modification of an Agreed Paediatric Investigation Plan

2.3.1. Elvitegravir - EMEA-000968-PIP02-11-M05

Gilead Sciences International Ltd; Human immunodeficiency virus [HIV] disease resulting in other conditions [ICD-10: B23] / indicated for use with a pharmacoenhancer and other antiretroviral agents for the treatment of HIV-1 infection in paediatric patients aged < 18 years.

Day 60 opinion

Action: For adoption

Infectious Diseases

2.3.2. apixaban - EMEA-000183-PIP01-08-M04

Bristol-Myers Squibb / Pfizer EEIG; Prevention of arterial thromboembolism, Prevention of venous thromboembolism / Prevention of venous thromboembolism (VTE) in paediatric subjects (1 to <18 years old) with a newly diagnosed acute lymphoblastic leukemia (ALL) or lymphoma (T or B cell), a functioning central venous access device (CVAD) and receiving PEG L-asparaginase during chemotherapy induction, Prevention of TE in paediatric patients (birth to below 18 years old) with cardiac disease

Day 60 opinion

Action: For adoption

Cardiovascular Diseases

2.3.3. selepressin - EMEA-000506-PIP01-08-M02

Ferring Pharmaceuticals A/S; Septic shock / Vasopressor-dependent Septic Shock

Day 60 opinion

Action: For adoption

Cardiovascular Diseases

2.3.4. albiglutide - EMEA-001175-PIP01-11-M04

Glaxo Group Limited; Non-insulin dependent diabetes mellitus / type 2 diabetes mellitus

Day 60 opinion

Action: For adoption

Endocrinology-Gynaecology-Fertility-Metabolism

2.3.5. Alogliptin benzoate (as alogliptin) - EMEA-000496-PIP01-08-M05

Takeda Development Centre Europe Ltd; Type 2 diabetes melitus

Day 60 opinion

Action: For adoption

Endocrinology-Gynaecology-Fertility-Metabolism

2.3.6. Sapropterin Dihydrochloride - Orphan - EMEA-001476-PIP01-13-M01

BioMarin International Limited; Hyperphenylalaninemia / BH4 deficiency, Phenylketonuria

Day 60 opinion

Action: For adoption

Endocrinology-Gynaecology-Fertility-Metabolism

2.3.7. eftrenonacog alfa - Orphan - EMEA-000914-PIP01-10-M03

Biogen Idec Ltd; Hereditary Factor IX Deficiency - D67

Day 60 opinion

Action: For adoption

Haematology-Hemostaseology

2.3.8. Autologous CD34+ cells transduced with lentiviral vector containing the human Wiskott Aldrich Syndrom Protein gene - Orphan - EMEA-000786-PIP01-09-M02

Genethon; Treatment of Wiskott-Aldrich syndrome

Day 60 opinion

Action: For adoption

Immunology-Rheumatology-Transplantation

2.3.9. Delamanid - Orphan - EMEA-001113-PIP01-10-M05

Otsuka Europe Development and Commercialisation Ltd.; Treatment of multi drug resistant tuberculosis / Treatment of multi drug resistant tuberculosis

Day 60 opinion

Action: For adoption

Infectious Diseases

2.3.10. Tedizolid phosphate - EMEA-001379-PIP01-12-M02

Merck Sharp & Dohme (Europe), Inc.; Treatment of complicated skin and soft tissue infections / Treatment of complicated skin and soft tissue infections

Day 60 opinion

Action: For adoption

Infectious Diseases

2.3.11. nusinersen - Orphan - EMEA-001448-PIP01-13-M02

Ionis Pharmaceuticals, Inc.; Spinal muscular atrophy

Day 60 opinion

Action: For adoption

Neurology

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

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

Day 60 opinion

Action: For adoption

Neurology

2.3.13. zuretinol acetate - Orphan - EMEA-001453-PIP01-13-M01

QLT Ophthalmics (UK), Ltd.; Retinitis Pigmentosa, Leber Congenital Amaurosis / Treatment of patients with Inherited Retinal Disease who have been phenotypically diagnosed as LCA or RP caused by mutations in retinal pigment epithelium protein 65 (RPE65) or lecithin: retinol acyltransferase (LRAT) genes

Day 60 opinion

Action: For adoption

Ophthalmology

2.3.14. Human recombinant IgG1 monoclonal antibody targeting fibroblast growth factor 23 (FGF23) - Orphan - EMEA-001659-PIP01-15-M01

Ultragenyx Pharmaceutical Inc.; X-linked Hypophosphatemia

Day 60 opinion

Action: For adoption

Other

2.3.15. Human Thrombin (component 2) / Human Fibrinogen (component 1) - EMEA-001598-PIP01-13-M02

Instituto Grifols, S.A.; Treatment of haemorrhage resulting from a surgical procedure /

Supportive treatment in surgery where standard surgical techniques are insufficient for improvement of haemostasis, and as a suture support in vascular surgery

Day 60 opinion

Action: For adoption

Other

2.3.16. mepolizumab - Orphan - EMEA-000069-PIP02-10-M06

GSK Trading Services Limited; treatment of asthma / add-on treatment for severe refractory eosinophilic asthma

Day 60 opinion

Action: For adoption

Pneumology - Allergology

2.3.17. Peanut flour - EMEA-001734-PIP01-14-M01

Aimmune Therapeutics; Peanut Allergy / Peanut oral immunotherapy for the reduction in clinical reactivity to accidental exposure in peanut allergic children and adults

Day 60 opinion

Action: For adoption

Pneumology - Allergology

2.3.18. Reslizumab - EMEA-001202-PIP02-13-M01

Teva Pharmaceuticals Europe; Treatment of asthma / indicated as add−on treatment in adult patients with severe eosinophilic asthma

Day 60 opinion

Action: For adoption

Pneumology - Allergology

2.3.19. EMEA-000431-PIP01-08-M09

Glaxo Group Limited; Mixed Asthma / Treatment of asthma where use of a combination product (long acting beta agonist and inhaled corticosteroid) is appropriate

Day 60 opinion

Action: For adoption

Pneumology - Allergology

2.4. Opinions on Re-examinations

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

Eli Lilly & Company; Type 2 diabetes mellitus

Day 30 opinion

Action: For adoption

Endocrinology-Gynaecology-Fertility-Metabolism

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

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

Day 30 opinion

Action: For adoption

Other

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. blisibimod - EMEA-001972-PIP01-16

systemic lupus erythematosus

Day 60 discussion

Action: For discussion

Immunology-Rheumatology-Transplantation

3.1.2. 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 90 discussion

Action: For discussion

Gastroenterology-Hepatology

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

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

Day 90 discussion

Action: For discussion

Infectious Diseases

3.1.4. Immunoglobulin G2, anti-(human α-calcitonin gene-related peptide/β-calcitonin gene-related peptide) (human-Mus musculus monoclonal TEV-48125 heavy chain), disulphide with human-Mus musculus monoclonal TEV-48125 light chain, dimer - 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 90 discussion

Action: For discussion

Neurology

3.1.5. 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 90 discussion

Action: For discussion

Oncology

3.1.6. 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 90 discussion

Action: For discussion

Pneumology - Allergology

3.1.7. 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 90 discussion

Action: For discussion

Vaccines

3.1.8. Human bone marrow-derived allogeneic mesenchymal precursor cells (MPCs) - EMEA-001827-PIP02-16

chronic heart failure

Day 60 discussion

Action: For discussion

Cardiovascular Diseases

3.1.9. Allogeneic, non-expanded, umbilical Cord blood-derived, hematopoietic mature myeloid and lymphoid cells (NF) / Allogeneic, ex vivo expanded, umbilical Cord blood-derived, hematopoietic CD34+ progenitor cells (CF) - Orphan - EMEA-001913-PIP01-15

Gamida Cell Limited; acute lymphoblastic leukaemia, myelodysplastic syndrome, acute myeloid leukaemia, chronic myeloid leukaemia / haematopoietic reconstitution of patients who are medically indicated for allogeneic haematopoietic stem cell transplantation

Day 60 discussion

Action: For discussion

Haematology-Hemostaseology

3.1.10. EMEA-001741-PIP02-16

Treatment of Ulcerative Colitis

Day 60 discussion

Action: For discussion

Immunology-Rheumatology-Transplantation

3.1.11. Autologous CD34+ hematopoietic stem cells transduced ex vivo with EFS lentiviral vector encoding for the human ADA gene - Orphan - EMEA-001974-PIP01-16

Pr Bobby Gaspar; Severe combined immunodeficiency disorder due to adenosine deaminase deficiency [ADA-SCID] / Treatment of severe combined immunodeficiency disorder due to adenosine deaminase deficiency [ADA-SCID]

Day 60 discussion

Action: For discussion

Immunology-Rheumatology-Transplantation

3.1.12. T-lymphocytes enriched leukocyte preparation depleted ex vivo of host host-alloreactive T cells using photodynamic treatment - Orphan - EMEA-001980-PIP01-16

Kiadis Pharma Netherlands B.V.; Adjunctive treatment in haematopoietic stem cell transplantation for a malignant disease / Adjunctive treatment to a haploidentical haematopoietic stem cell transplantation with CD34+ selected cells, in patients with a haematological malignancy, for the reduction of morbidity (i.e. incidences and severity of graft versus host disease) and mortality due to infection and relapse

Day 60 discussion

Action: For discussion

Immunology-Rheumatology-Transplantation / Oncology

3.1.13. EMEA-001975-PIP01-16

Treatment of influenza

Day 60 discussion

Action: For discussion

Infectious Diseases

3.1.14. (Eubacterial Spores, Purified Suspension, Encapsulated) - EMEA-001970-PIP01-16

ICD10 code A04.7: Enterocolitis due to Clostridium difficile / indicated as a treatment, at the completion of antibiotic therapy, of paediatric patients with active recurrent Clostridium difficile infection to prevent further recurrence

Day 60 discussion

Action: For discussion

Infectious Diseases

3.1.15. Fevipiprant - EMEA-001315-PIP02-16

Asthma / Treatment of moderate to severe asthma

Day 60 discussion

Action: For discussion

Pneumology - Allergology

3.1.16. olodaterol hydrochloride - EMEA-001965-PIP01-16

Treatment of cystic fibrosis

Day 60 discussion

Action: For discussion

Pneumology - Allergology

3.1.17. Live attenuated, chimeric dengue virus, serotype 4 / Live, attenuated, chimeric dengue virus, serotype 3 / Live, attenuated dengue virus, serotype 2 / Live, attenuated, chimeric dengue virus, serotype 1 - EMEA-001888-PIP01-15

Prevention of dengue fever

Day 60 discussion

Action: For discussion

Vaccines

3.1.18. Amlodipine / Candesartan - EMEA-002014-PIP01-16

Hypertension

Day 30 discussion

Action: For discussion

Cardiovascular Diseases

3.1.19. Amlodipine / Perindopril - EMEA-001968-PIP01-16

Hypertension

Day 30 discussion

Action: For discussion

Cardiovascular Diseases

3.1.20. 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 30 discussion

Action: For discussion

Cardiovascular Diseases

3.1.21. EMEA-001983-PIP01-16

Monitoring of renal function

Day 30 discussion

Action: For discussion

Diagnostic / Uro-nephrology

3.1.22. 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 30 discussion

Action: For discussion

Endocrinology-Gynaecology-Fertility-Metabolism

3.1.23. 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 30 discussion

Action: For discussion

Endocrinology-Gynaecology-Fertility-Metabolism / Cardiovascular Diseases

3.1.24. Amiselimod - EMEA-001991-PIP01-16

Ulcerative colitis / Treatment of moderately to severely active ulcerative colitis

Day 30 discussion

Action: For discussion

Gastroenterology-Hepatology

3.1.25. Cenicriviroc mesylate - EMEA-001999-PIP01-16

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

Day 30 discussion

Action: For discussion

Gastroenterology-Hepatology

3.1.26. Human fibrinogen concentrate - EMEA-001931-PIP01-16

Treatment of congenital fibrinogen deficiency

Day 30 discussion

Action: For discussion

Haematology-Hemostaseology

3.1.27. Pomalidomide - Orphan - EMEA-001457-PIP02-16

Celgene Europe Limited; Treatment of multiple myeloma / in combination with dexamethasone is indicated in the treatment of adult patients with relapsed and refractory multiple myeloma who have received at least two prior treatment regimens, including both lenalidomide and bortezomib, and have demonstrated disease progression on the last therapy

Day 30 discussion

Action: For discussion

Haematology-Hemostaseology

3.1.28. Sirukumab - EMEA-001043-PIP02-16

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

Day 30 discussion

Action: For discussion

Immunology-Rheumatology-Transplantation

3.1.29. 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)

Day 30 discussion

Action: For discussion

Infectious Diseases

3.1.30. 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 (eg, diffuse large B-cell lymphoma [DLBCL], Burkitt lymphoma [BL] and primary mediastinal B-cell lymphoma [PMBCL])

Day 30 discussion

Action: For discussion

Oncology

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

Halozyme Inc.; Pancreas cancer

Day 30 discussion

Action: For discussion

Oncology

3.1.32. Pexidartinib - Orphan - EMEA-001939-PIP02-16

Daiichi Sankyo Europe GmbH; Treatment of benign soft tissue neoplasm

Day 30 discussion

Action: For discussion

Oncology

3.1.33. Ciclosporin - EMEA-001998-PIP01-16

Dry eye disease/Keratoconjuntivitis Sicca

Day 30 discussion

Action: For discussion

Ophthalmology

3.1.34. Fluocinolone Acetonide - Orphan - EMEA-000801-PIP02-16

CAMPHARM Limited; Chronic non-infectious uveitis

Day 30 discussion

Action: For discussion

Ophthalmology

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

Disc degeneration disease / Not applicable, a full product-specific waiver for all paediatric subsets is sought

Day 30 discussion

Action: For discussion

Other

3.1.36. triheptanoin - Orphan - EMEA-001920-PIP01-15

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

Day 30 discussion

Action: For discussion

Other

3.1.37. Orphan - EMEA-001984-PIP01-16

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

Day 30 discussion

Action: For discussion

Uro-nephrology

3.1.38. Gentamicin sulphate - EMEA-001982-PIP01-16

Treatment of diabetic foot ulcers

Day 1 discussion

Action: For discussion

Dermatology/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. ertugliflozin - EMEA-C1-001533-PIP01-13

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

Day 30 discussion

Action: For discussion

Endocrinology-Gynaecology-Fertility-Metabolism

3.2.2. exanatide - EMEA-C1-000689-PIP01-09-M06

AstraZeneca AB; Treatment of type 2 diabetes mellitus

Day 30 discussion

Action: For discussion

Endocrinology-Gynaecology-Fertility-Metabolism

3.2.3. deferasirox - EMEA-C5-001103-PIP01-10-M03

Novartis Europharm Limited; Treatment of chronic iron overload requiring chelation therapy

Day 30 discussion

Action: For discussion

Haematology-Hemostaseology

3.2.4. raxibacumab - EMEA-C1-001569-PIP01-13

GlaxoSmithKline Trading Services Limited; Treatment of bacillary infection

Day 30 discussion

Action: For discussion

Infectious Diseases

3.2.5. Sofosbuvir / ledipasvir - EMEA-C1-001411-PIP01-12-M03

Gilead Sciences International Ltd.; Treatment of chronic hepatitis C

Day 30 discussion

Action: For discussion

Infectious Diseases

3.2.6. rufinamide - EMEA-C-000709-PIP01-09-M05

Eisai Limited; Treatment of Lennox-Gastaut Syndrome

Day 30 discussion

Action: For discussion

Neurology

3.2.7. Tralokinumab - EMEA-C1-000782-PIP01-09-M03

MedImmune Ltd; Treatment of asthma

Day 30 discussion

Action: For discussion

Pneumology - Allergology

3.3. Discussions on Modification of an Agreed Paediatric Investigation Plan

3.3.1. Alipogene Tiparvovec - Orphan - EMEA-000292-PIP01-08-M03

uniQure biopharma B.V.; Hyperchylomicronaemia

Day 30 discussion

Action: For discussion

Cardiovascular Diseases

3.3.2. 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 30 discussion

Action: For discussion

Cardiovascular Diseases

3.3.3. 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 30 discussion

Action: For discussion

Cardiovascular Diseases

3.3.4. 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 30 discussion

Action: For discussion

Cardiovascular Diseases / Haematology-Hemostaseology

3.3.5. Sodium zirconium cyclosilicate - EMEA-001539-PIP01-13-M01

ZS Pharma, Inc; Hyperkalaemia / Treatment of Hyperkalaemia

Day 30 discussion

Action: For discussion

Endocrinology-Gynaecology-Fertility-Metabolism

3.3.6. 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 30 discussion

Action: For discussion

Endocrinology-Gynaecology-Fertility-Metabolism

3.3.7. 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 30 discussion

Action: For discussion

Haematology-Hemostaseology

3.3.8. 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 30 discussion

Action: For discussion

Immunology-Rheumatology-Transplantation / Haematology-Hemostaseology

3.3.9. 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 30 discussion

Action: For discussion

Neurology

3.3.10. eteplirsen - Orphan - EMEA-001722-PIP01-14-M01

Sarepta International C.V.; Duchenne muscular dystrophy

Day 30 discussion

Action: For discussion

Neurology

3.3.11. 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 30 discussion

Action: For discussion

Oncology

3.3.12. Cenegermin - Orphan - EMEA-001729-PIP01-14-M01

Dompé farmaceutici S.p.A.; Neurotrophic Keratitis

Day 30 discussion

Action: For discussion

Ophthalmology

3.3.13. 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 30 discussion

Action: For discussion

Other

3.3.14. 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 30 discussion

Action: For discussion

Other / Haematology-Hemostaseology

3.3.15. ataluren - Orphan - EMEA-000115-PIP02-09-M03

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

Day 30 discussion

Action: For discussion

Pneumology - Allergology

3.3.16. 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 30 discussion

Action: For discussion

Pneumology - Allergology

3.3.17. Modified allergen extract of birch pollen - EMEA-000932-PIP01-10-M01

ROXALL Medizin GmbH; H10.1 Acute atopic conjunctivitis, J30.1 Allergic rhinitis due to pollen / Treatment of allergic rhinitis due to pollen of the birch group, Treatment of acute atopic conjunctivitis due to tree pollen of the birch group

Day 30 discussion

Action: For discussion

Pneumology - Allergology

3.3.18. 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 30 discussion

Action: For discussion

Uro-nephrology

3.3.19. 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 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 18 October 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

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. Brexpiprazole – EMEA-24-2016

Treatment of Alzheimer's Disease/ Treatment of agitation associated with dementia of the Alzheimer's type

Action: For adoption

6.1.2. Palucorcel - EMEA-25-2016

All classes of medicinal products for the treatment of age-related macular degeneration and diabetic macular oedema/ Improvement of visual acuity in patients with vision loss from geographic atrophy secondary to age-related macular degeneration

Action: For adoption

6.1.3. BKM120 (buparlisib) - EMEA-26-2016

Treatment of oropharyngeal, laryngeal or nasal epithelial carcinoma (excluding nasopharyngeal carcinoma or lymphoepithelioma)/ Treatment of patients with recurrent or metastatic head and neck squamous cell carcinoma (HNSCC)

Action: For adoption

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

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

None

8. Annual reports on deferrals

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

9. Organisational, regulatory and methodological matters

9.1. Mandate and organisation of the PDCO

9.1.1. Proposals for optimisation of PDCO plenary meetings

Action: For information

9.1.2. Criteria for expertise and experience of PDCO members

Action: For adoption

9.1.3. Preparations for elections of PDCO Chair

Action: For information

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. CHMP-PDCO Interaction

PDCO Chair: Dirk Mentzer

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

None

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. None.

11. Breakout sessions

11.1.1. Paediatric oncology

Action: For discussion on Thursday, 18:30 – 17:30, room 3M

11.1.2. Neonatology

Action: For discussion on Thursday, 18:30 – 17:30, 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/