23 July 2019
EMA/PDCO/380845/2019
Inspections, Human Medicines Pharmacovigilance and Committees Division

Paediatric Committee (PDCO)

Draft agenda for the meeting on 23-26 July 2019

Chair: Dirk Mentzer – Vice-Chair: Koenraad Norga

23 July 2019, 14:00- 19:00, room 2D

24 July 2019, 08:30- 19:00, room 2D

25 July 2019, 08:30- 19:00, room 2D

26 July 2019, 08:30- 13:00, room 2D

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 23-26 July 2019. See 23-26 July 2019 PDCO minutes (to be published post 17-20 September 2019 PDCO meeting).

1.2. Adoption of agenda

PDCO agenda for 23-26 July 2019

1.3. Adoption of the minutes

PDCO minutes for 25-28 June 2019

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. Birch bark extract - Orphan - EMEA-001299-PIP03-17

Day 120 Opinion

Action: For adoption

Dermatology

2.1.2. Trifarotene cream HE1 - EMEA-001492-PIP02-18

Lamellar Ichthyosis / Treatment of lamellar ichthyosis

Day 120 Opinion

Action: For adoption

Dermatology

2.1.3. Levonorgestrel - EMEA-002474-PIP02-18

Contraception

Day 120 Opinion

Action: For adoption

Endocrinology-Gynaecology-Fertility-Metabolism

2.1.4. Tirzepatide - EMEA-002360-PIP01-18

Type 2 diabetes mellitus / Treatment of type 2 diabetes mellitus

Day 120 Opinion

Action: For adoption

Endocrinology-Gynaecology-Fertility-Metabolism

2.1.5. EMEA-002448-PIP01-18

Treatment of non-alcoholic steatohepatitis (NASH) / Treatment of NASH with moderate to severe liver fibrosis

Day 120 Opinion

Action: For adoption

Gastroenterology-Hepatology

2.1.6. Bimekizumab - EMEA-002189-PIP02-18

Treatment of chronic idiopathic arthritis (including rheumatoid arthritis, spondyloarthritis, psoriatic arthritis and juvenile idiopathic arthritis (JIA)) / Treatment of JIA (enthesitis-related arthritis (ERA) and juvenile psoriatic arthritis (JPsA)) in patients from ≥2 years to <18 years of age

Day 120 Opinion

Action: For adoption

Immunology-Rheumatology-Transplantation

2.1.7. Liposomal ciclosporin A (L-CsA) - Orphan - EMEA-002344-PIP02-18

Breath Therapeutics GmbH; Treatment of bronchiolitis obliterans syndrome (BOS)

Day 120 Opinion

Action: For adoption

Immunology-Rheumatology-Transplantation

2.1.8. Baloxavir marboxil - EMEA-002440-PIP01-18

Prevention of influenza / Treatment of influenza / Treatment of influenza type A/B in otherwise healthy, high risk and hospitalised patients / Prevention (post-exposure prophylaxis) of influenza type A/B. Reduction of transmission of influenza type A/B

Day 120 Opinion

Action: For adoption

Infectious Diseases

2.1.9. Equine Immunoglobulin F(ab')2 fragments targeting Shiga toxin - Orphan - EMEA-002444-PIP02-18

Chemo Research, S.L.; Prevention of Shiga-toxin producing Escherichia coli haemolytic uremic syndrome

Day 120 Opinion

Action: For adoption

Infectious Diseases

2.1.10. Eptinezumab - EMEA-002243-PIP01-17

Prevention of migraine headaches / Prophylaxis of migraine

Day 120 Opinion

Action: For adoption

Neurology

2.1.11. Fosmetpantotenate - Orphan - EMEA-002036-PIP01-16

Retrophin Europe Limited; Treatment of pantothenate kinase associated neurodegeneration (PKAN)

Day 120 Opinion

Action: For adoption

Neurology

2.1.12. Niraparib (as tosylate monohydrate) - Orphan - EMEA-002268-PIP02-18

Tesaro UK Ltd; Treatment of all conditions included in the category of malignant neoplasms (except haematopoietic and lymphoid malignancies) / The combination therapy is for the treatment of patients ≥6 months to <18 years of age who have been diagnosed with recurrent solid tumours that exhibit a breast cancer susceptibility gene (BRCA)ness mutational signature (mutational signature 3)

Day 120 Opinion

Action: For adoption

Oncology

2.1.13. Aldesleukin - EMEA-002492-PIP01-18

Treatment of all conditions included in the category of malignant neoplasms (except nervous system, haematopoetic, and lymphoid tissue) / In combination with nivolumab (or with nivolumab and ipilimumab) for the treatment of a relapsed or refractory paediatric malignant solid tumour in paediatric patients less than 18 years old / In combination with nivolumab (or with nivolumab and ipilimumab) for the treatment of patients with unresectable or metastatic melanoma in the age group from 12 to less than 18 years old

Day 120 Opinion

Action: For adoption

Oncology

2.1.14. Pegvorhyaluronidase alfa - Orphan - EMEA-001883-PIP03-17

Halozyme Inc; Treatment of solid malignant tumours (except central nervous system tumours, haematopoietic and lymphoid tissue tumours) / Pegvorhyaluronidase alfa is indicated in combination with cytotoxic cancer therapies for the treatment of paediatric patients aged from birth to less than 18 years with relapsed or refractory solid tumours that accumulate high levels of hyaluronan

Day 120 Opinion

Action: For adoption

Oncology

2.1.15. Anti PD-1 monoclonal antibody - EMEA-002463-PIP01-18

Treatment of all conditions included in the category of malignant neoplasms (except haematopoietic and lymphoid malignancies) / The combination therapy is for the treatment of patients ≥6 months to <18 years of age who have been diagnosed with recurrent solid tumours that exhibit a breast cancer susceptibility gene (BRCA)ness mutational signature (mutational signature 3)

Day 120 Opinion

Action: For adoption

Oncology

2.1.16. Nebivolol / zofenopril - EMEA-002593-PIP01-19

Treatment of essential hypertension

Day 60 Opinion

Action: For adoption

Cardiovascular Diseases

2.1.17. Ramipril / rosuvastatin - EMEA-002569-PIP01-19

Treatment of cardiovascular disease

Day 60 Opinion

Action: For adoption

Cardiovascular Diseases

2.1.18. Belantamab mafodotin - Orphan - EMEA-002468-PIP04-19

GlaxoSmithKline Trading Services; Treatment of multiple myeloma

Day 60 Opinion

Action: For adoption

Oncology

2.1.19. Infigratinib - EMEA-002594-PIP01-19

Cholangiocarcinoma

Day 60 Opinion

Action: For adoption

Oncology

2.1.20. Difelikefalin - EMEA-002565-PIP01-19

Chronic kidney disease (CKD)-associated pruritus

Day 60 Opinion

Action: For adoption

Other

2.1.21. Obidoxime chloride / atropine sulfate - EMEA-002570-PIP01-19

Organophosphate nerve agent poisoning

Day 60 Opinion

Action: For adoption

Other

2.1.22. Fexapotide triflutate - EMEA-002598-PIP01-19

Treatment of patients with benign prostatic hyperplasia (BPH)

Day 60 Opinion

Action: For adoption

Uro-nephrology

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. Etravirine - EMEA-C-000222-PIP01-08-M09

Janssen-Cilag International NV; Treatment of human immunodeficiency virus (HIV-1) infection

Day 60 Opinion

Action: For adoption

Infectious Diseases

2.3. Opinions on Modification of an Agreed Paediatric Investigation Plan

2.3.1. Sacubitril / valsartan - EMEA-000316-PIP02-11-M04

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

Day 60 Opinion

Action: For adoption

Cardiovascular Diseases

2.3.2. Ticagrelor - EMEA-000480-PIP01-08-M12

AstraZeneca AB; Thromboembolic events (children), acute coronary syndrome, history of myocardial infarction / Reduction in occurrence of vaso-occlusive crises in paediatric patients with sickle cell disease

Day 60 Opinion

Action: For adoption

Cardiovascular Diseases / Haematology-Hemostaseology

2.3.3. Liquid ethanolic extract 30 per cent (w/w) of Allium cepa L. (fresh bulb) and Citrus limon (L.) Burm. f. (fresh fruit), Paullinia cupana Kunth, Theobroma cacao L. - EMEA-001835-PIP01-15-M04

LEGACY HEALTHCARE; Treatment of alopecia

Day 60 Opinion

Action: For adoption

Dermatology

2.3.4. 2-hydroxypropyl-ß-cyclodextrin (HP-ß-CD) - Orphan - EMEA-001866-PIP01-15-M04

Mallinckrodt Pharmaceuticals Ireland Ltd; Treatment of Niemann-Pick disease, type C

Day 60 Opinion

Action: For adoption

Endocrinology-Gynaecology-Fertility-Metabolism

2.3.5. Empagliflozin - EMEA-000828-PIP04-16-M03

Boehringer Ingelheim International GmbH; Treatment of type 1 diabetes mellitus

Day 60 Opinion

Action: For adoption

Endocrinology-Gynaecology-Fertility-Metabolism

2.3.6. Semaglutide - EMEA-001441-PIP03-17-M01

Novo Nordisk A/S; Treatment of Obesity

Day 60 Opinion

Action: For adoption

Endocrinology-Gynaecology-Fertility-Metabolism

2.3.7. Etrolizumab - EMEA-001434-PIP01-13-M02

Roche Registration GmbH; Treatment of ulcerative colitis / Treatment of Crohn's disease / Treatment of moderately to severely active Crohn's disease / Treatment of moderately to severely active ulcerative colitis

Day 60 Opinion

Action: For adoption

Gastroenterology-Hepatology

2.3.8. Odevixibat - Orphan - EMEA-002054-PIP01-16-M01

Albireo AB; Progressive familial intrahepatic cholestasis (PFIC)

Day 60 Opinion

Action: For adoption

Gastroenterology-Hepatology

2.3.9. Ferric maltol - EMEA-001195-PIP01-11-M04

Norgine BV; Iron deficiency anaemia / Treatment for iron deficiency anaemia (IDA)

Day 60 Opinion

Action: For adoption

Haematology-Hemostaseology

2.3.10. Lonoctocog alfa - EMEA-001215-PIP01-11-M07

CSL Behring GmbH; Haemophila A

Day 60 Opinion

Action: For adoption

Haematology-Hemostaseology

2.3.11. Upadacitinib - EMEA-001741-PIP01-14-M02

AbbVie Ltd; Treatment of chronic idiopathic arthritis / Treatment of juvenile idiopathic arthritis

Day 60 Opinion

Action: For adoption

Immunology-Rheumatology-Transplantation

2.3.12. Ustekinumab - EMEA-000311-PIP03-11-M05

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

Day 60 Opinion

Action: For adoption

Immunology-Rheumatology-Transplantation

2.3.13. Lamivudine (3TC) / dolutegravir (DTG) - EMEA-001940-PIP01-16-M02

ViiV Healthcare BV; Treatment of human immunodeficiency virus (HIV-1) infection

Day 60 Opinion

Action: For adoption

Infectious Diseases

2.3.14. Nirsevimab - anti-respiratory syncytial virus human IgG1κ monoclonal antibody - EMEA-001784-PIP01-15-M01

AstraZeneca AB; Prevention of respiratory syncytial viral infections

Day 60 Opinion

Action: For adoption

Infectious Diseases

2.3.15. Pretomanid - Orphan - EMEA-002115-PIP01-17-M01

Global Alliance for TB Drug Development; Treatment of multi-drug-resistant tuberculosis

Day 60 Opinion

Action: For adoption

Infectious Diseases

2.3.16. Arimoclomol citrate - Orphan - EMEA-001748-PIP01-15-M02

Orphazyme A/S; Treatment of Niemann-Pick Disease, type C

Day 60 Opinion

Action: For adoption

Neurology

2.3.17. Ataluren - Orphan - EMEA-000115-PIP01-07-M10

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

Day 60 Opinion

Action: For adoption

Neurology

2.3.18. Brivaracetam - Orphan - EMEA-000332-PIP01-08-M14

UCB Pharma S.A.; Treatment of neonatal seizures / Treatment of epilepsy with partial onset seizures / Treatment of paediatric patients with partial onset seizures / Treatment of neonatal seizures with adjunctive administration of brivaracetam

Day 60 Opinion

Action: For adoption

Neurology

2.3.19. Onasemnogene abeparvovec - Orphan - EMEA-002168-PIP01-17-M02

AveXis Netherlands B.V.; Treatment of spinal muscular atrophy / Treatment of spinal muscular atrophy type 1

Day 60 Opinion

Action: For adoption

Neurology

2.3.20. Risdiplam - Orphan - EMEA-002070-PIP01-16-M03

Roche Registration GmbH; Treatment of spinal muscular atrophy

Day 60 Opinion

Action: For adoption

Neurology

2.3.21. Cabozantinib - Orphan - EMEA-001143-PIP01-11-M02

Ipsen Pharma; Treatment of malignant solid tumours

Day 60 Opinion

Action: For adoption

Oncology

2.3.22. Cobimetinib - EMEA-001425-PIP01-13-M04

Roche Registration GmbH; Treatment of all conditions included in the category of malignant neoplasms (except haematopoietic and lymphoid tissue) with Ras, Raf or MEK pathway activation / Treatment of children with a paediatric solid malignant tumour with known or expected Ras, Raf or MEK pathway activation, at first relapse or refractory to initial treatment.

Day 60 Opinion

Action: For adoption

Oncology

2.3.23. Larotrectinib - Orphan - EMEA-001971-PIP02-16-M02

Bayer AG; Treatment of all conditions included in the category of malignant neoplasms (except central nervous system tumours, haematopoietic and lymphoid tissue neoplasms) / Treatment of paediatric patients from birth to less than 18 years of age with advanced solid tumours harbouring an NTRK fusion

Day 60 Opinion

Action: For adoption

Oncology

2.3.24. Pazopanib - EMEA-000601-PIP01-09-M06

Novartis Europharm Limited; Ewing sarcoma family of tumours / Rhabdomyosarcoma / Non-rhabdomyosarcoma soft tissue sarcoma / Treatment of pediatric patients with rhabdomyosarcoma / Treatment of pediatric patients with Ewing sarcoma family of tumours / Treatment of pediatric patients with non-rhabdomyosarcoma soft tissue sarcoma

Day 60 Opinion

Action: For adoption

Oncology

2.3.25. Tisagenlecleucel - Orphan - EMEA-001654-PIP02-17-M01

Novartis Europharm Limited; Mature B-cell neoplasm / Treatment of paediatric patients with CD19+ relapsed or refractory mature B-cell non-Hodgkin's lymphoma

Day 60 Opinion

Action: For adoption

Oncology

2.3.26. Human thrombin / human fibrinogen - EMEA-001149-PIP01-11-M05

Omrix Biopharmaceuticals N.V.; Treatment of cerebrospinal fluid leakage resulting from a surgical procedure / Treatment of haemorrhage resulting from a surgical procedure / Indicated for suture line sealing in dura mater closure / Indicated for supportive treatment in surgery where standard surgical techniques are insufficient, for improvement of haemostasis/ Indicated for supportive treatment in surgery where standard surgical techniques are insufficient, for improvement of haemostasis.

Day 60 Opinion

Action: For adoption

Other

2.3.27. Lomitapide - EMEA-001124-PIP01-10-M04

Amryt Pharmaceuticals DAC; Treatment of heterozygous and homozygous familial hypercholesterolaemia / Treatment of homozygous familial hypercholesterolaemia / Treatment of heterozygous familial hypercholesterolemia

Day 60 Opinion

Action: For adoption

Other

2.3.28. Dermatophagoides farinae / Dermatophagoides pteronyssinus - EMEA-001258-PIP01-11-M05

ALK-Abelló A/S; Treatment of allergic rhinitis / Treatment of asthma / Indicated in house dust mite allergic asthma / Indicated in house dust mite allergic rhinitis

Day 60 Opinion

Action: For adoption

Pneumology - Allergology

2.3.29. Fevipiprant - EMEA-001315-PIP02-16-M02

Novartis EuroPharm Limited; Asthma / Treatment of uncontrolled persistent asthma

Day 60 Opinion

Action: For adoption

Pneumology - Allergology

2.3.30. Finerenone - EMEA-001623-PIP01-14-M03

Bayer AG; Chronic kidney disease / Treatment of chronic kidney disease associated with proteinuria in addition to a therapy with angiotensin converting enzyme inhibitor (ACEi) or angiotensin receptor blocker (ARB)

Day 60 Opinion

Action: For adoption

Uro-nephrology

2.3.31. Influenza virus surface antigens (haemagglutinin) of strain A (H1N1) / influenza virus surface antigens (haemagglutinin) of strain A (H3N2) / influenza virus surface antigens (haemagglutinin) of strain B (Victoria lineage) / influenza virus surface antigens (haemagglutinin) of strain B (Yamagata lineage) - EMEA-002027-PIP02-17-M01

Adimmune Corporation; Prevention of influenza infection

Day 60 Opinion

Action: For adoption

Vaccines

2.3.32. Outer membrane vescicles (OMV) from neisseria meningitidis serogroup B strain NZ98/254 measured as amount of total protein containing the PorA P1.4 / Recombinant neisseria meningitidis serogroup B fHbp fusion protein / Recombinant neisseria meningitidis serogroup B NadA protein / Recombinant neisseria meningitidis serogroup B NHBA fusion protein - EMEA-000139-PIP01-07-M03

GSK Vaccines S.r.l.; Prevention of meningococcal meningitis

Day 60 Opinion

Action: For adoption

Vaccines

2.3.33. Prepandrix: A/Indonesia/05/2005 (H5N1) like strain used / Adjupanrix: split influenza virus, inactivated, containing antigen: A/VietNam/1194/2004 (H5N1) like strain used - EMEA-000160-PIP01-07-M05

GlaxoSmithKline Biologicals SA; Prevention of influenza infection

Day 60 Opinion

Action: For adoption

Vaccines

2.4. Opinions on Re-examinations

No items

2.5. Opinions on Review of Granted Waivers

No items

2.6. Finalisation and adoption of Opinions

No items

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. Quizartinib - EMEA-C3-001821-PIP01-15-M03

Daiichi Sankyo Europe GmbH; Treatment of acute myeloid leukaemia

Day 1 letter

Action: For information

Oncology

2.7.2. Peanut (Arachis hypogaea) allergens (previously known as Peanut flour) - EMEA-C2-001734-PIP01-14-M04

Aimmune Therapeutics Netherlands B.V.; Treatment of peanut allergy

Day 1 letter

Action: For information

Pneumology – Allergology

2.7.3. Canagliflozin hemihydrate - EMEA-C1-001030-PIP01-10-M07

Janssen-Cilag International NV; Treatment of type 2 diabetes mellitus

Day 1 letter

Action: For information

Endocrinology-Gynaecology-Fertility-Metabolism

2.7.4. Satralizumab - EMEA-C1-001625-PIP01-14-M03

Roche Registration GmbH; Treatment of neuromyelitis optica

Day 1 letter

Action: For information

Neurology

2.8. Revision of PDCO Opinions

2.8.1. Recombinant adeno-associated viral vector serotype 2 carrying the gene for the human aromatic L-amino acid decarboxylase protein - Orphan - EMEA-002435-PIP01-18

PTC Therapeutic International Limited; Aromatic L-amino acid decarboxylase (AADC) deficiency / Treatment of aromatic L-amino acid decarboxylase (AADC) deficiency

Action: For adoption

Neurology

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. Dihomo-γ-linolenic acid (DGLA) - EMEA-002364-PIP03-19

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

Day 60 discussion

Action: For discussion

Dermatology

3.1.2. Lebrikizumab - EMEA-002536-PIP01-18

Treatment of atopic dermatitis

Day 60 discussion

Action: For discussion

Dermatology

3.1.3. Deoxycytidine - Orphan - EMEA-002513-PIP01-18

Modis Therapeutics, Inc.; Treatment of thymidine kinase 2 deficiency

Day 60 discussion

Action: For discussion

Endocrinology-Gynaecology-Fertility-Metabolism

3.1.4. Deoxythymidine - Orphan - EMEA-002624-PIP01-19

Modis Therapeutics, Inc.; Treatment of thymidine kinase 2 deficiency

Day 60 discussion

Action: For discussion

Endocrinology-Gynaecology-Fertility-Metabolism

3.1.5. Pegcetacoplan - Orphan - EMEA-002600-PIP01-19

Apellis Ireland Limited; Paroxysmal nocturnal haemoglobinuria

Day 60 discussion

Action: For discussion

Haematology-Hemostaseology

3.1.6. Baricitinib - EMEA-001220-PIP05-19

Treatment of systemic lupus erythematosus

Day 60 discussion

Action: For discussion

Immunology-Rheumatology-Transplantation

3.1.7. Mavorixafor - EMEA-002490-PIP01-18

Treatment of warts, hypogammaglobulinemia, infections, and myelokathexis (WHIM)

syndrome / Treatment of WHIM syndrome in paediatric patients aged 6 years and above

Day 60 discussion

Action: For discussion

Immunology-Rheumatology-Transplantation

3.1.8. Polymyxin B - EMEA-002595-PIP01-19

Treatment of infections due to aerobic Gram-negative bacteria

Day 60 discussion

Action: For discussion

Infectious Diseases

3.1.9. Zoliflodacin - EMEA-002599-PIP01-19

Treatment of gonococcal infection / Treatment of uncomplicated gonorrhoea

Day 60 discussion

Action: For discussion

Infectious Diseases

3.1.10. EMEA-002572-PIP01-19

Chromosome 15q duplication syndrome / Cyclin-dependent kinase-like 5 (CDKL5) deficiency disorder / Treatment of seizures associated with chromosome 15q duplication syndrome / Treatment of seizures associated with CDKL5 deficiency disorder

Day 60 discussion

Action: For discussion

Neurology

3.1.11. Adeno-associated viral vector serotype 8 containing the human RPGR gene - Orphan - EMEA-002601-PIP01-19

Nightstar Europa Limited; Treatment of X-linked retinitis pigmentosa

Day 60 discussion

Action: For discussion

Ophthalmology

3.1.12. Recombinant hepatitis B vaccine - EMEA-002157-PIP01-17

Prevention of infection of hepatitis B virus / Secondary immunization of non-responder (anti-HBs levels< 10 mIU/mL) or hypo-responder (anti-HBs levels 10-99 mIU/mL) children

age 2-18 years old to prior immunization against hepatitis B virus (HBV) infection / Primary active immunization of children from birth to 18 years old for the prevention of hepatitis B infection

Day 60 discussion

Action: For discussion

Vaccines

3.1.13. Aprocitentan - EMEA-001978-PIP02-19

Hypertension

Day 30 discussion

Action: For discussion

Cardiovascular Diseases

3.1.14. Botulinum toxin type A - EMEA-002628-PIP01-19

Post-operative atrial fibrillation in patients undergoing open-chest cardiac surgery

Day 30 discussion

Action: For discussion

Cardiovascular Diseases

3.1.15. EMEA-002608-PIP01-19

Prostate cancer - diagnosis

Day 30 discussion

Action: For discussion

Diagnostic / Oncology

3.1.16. Avatrombopag maleate - EMEA-001136-PIP02-19

Chemotherapy-induced thrombocytopenia / Treatment of chemotherapy-induced thrombocytopenia (CIT) in patients receiving myelosuppressive chemotherapy for solid tumours

Day 30 discussion

Action: For discussion

Haematology-Hemostaseology

3.1.17. Human fibrinogen - EMEA-001931-PIP02-19

Treatment of acquired fibrinogen deficiency

Day 30 discussion

Action: For discussion

Haematology-Hemostaseology

3.1.18. Marzeptacog alfa (activated) - Orphan - EMEA-002270-PIP01-19

Voisin Consulting S.A.R.L; Treatment of haemophilia B

Day 30 discussion

Action: For discussion

Haematology-Hemostaseology

3.1.19. Marzeptacog alfa (activated) - EMEA-002270-PIP02-19

Treatment of haemophilia A

Day 30 discussion

Action: For discussion

Haematology-Hemostaseology

3.1.20. Allogeneic, non-expanded, umbilical cord blood-derived, haematopoietic mature myeloid and lymphoid cells (NF[1]) / Allogeneic, ex vivo expanded, umbilical cord blood-derived, haematopoietic CD34+progenitor cells (CF[2]) - Orphan - EMEA-001913-PIP02-18

Gamida Cell Ltd; Treatment in haematopoietic stem cell transplantation / Haematopoietic reconstitution of patients who are medically indicated for allogeneic haematopoietic stem cell transplantation

Day 30 discussion

Action: For discussion

Immunology-Rheumatology-Transplantation

3.1.21. C1-esterase inhibitor human - Orphan - EMEA-002316-PIP03-19

CSL Behring GmbH; Treatment of antibody mediated rejection (AMR) in kidney transplantation

Day 30 discussion

Action: For discussion

Immunology-Rheumatology-Transplantation

[1] Non cultured fraction
[2] Cultured fraction

3.1.22. Ibrexafungerp citrate - EMEA-002535-PIP02-19

Vulvovaginal candidiasis

Day 30 discussion

Action: For discussion

Infectious Diseases

3.1.23. EMEA-002566-PIP01-19

Treatment of multiple sclerosis / Treatment of secondary progressive multiple sclerosis / Treatment of primary progressive multiple sclerosis / Treatment of relapsing remitting multiple sclerosis

Day 30 discussion

Action: For discussion

Neurology

3.1.24. EMEA-002424-PIP02-19

Lewy body dementia

Day 30 discussion

Action: For discussion

Neurology

3.1.25. Arimoclomol citrate - Orphan - EMEA-001748-PIP03-19

Orphazyme A/S; Treatment of amyloid lateral sclerosis

Day 30 discussion

Action: For discussion

Neurology

3.1.26. Cannabidiol - EMEA-001964-PIP02-19

Treatment of Rett Syndrome

Day 30 discussion

Action: For discussion

Neurology

3.1.27. Dexamethasone (sodium phosphate) encapsulated in human autologous erythrocytes - Orphan - EMEA-001957-PIP02-19

EryDel S.p.A; Treatment of ataxia telangiectasia (AT) / Treatment of neurological symptoms in patients with AT

Day 30 discussion

Action: For discussion

Neurology

3.1.28. Efgartigimod alfa - Orphan - EMEA-002597-PIP01-19

argenx BVBA; Treatment of myasthenia gravis

Day 30 discussion

Action: For discussion

Neurology

3.1.29. Adenovirus serotype 5 containing partial E1A deletion and an integrin-binding domain - Orphan - EMEA-002314-PIP01-17

DNAtrix, Inc.; High-Grade Glioma / Treatment of unresectable high-grade glioma in first recurrence, and diffuse intrinsic pontine glioma after failure of radiotherapy

Day 30 discussion

Action: For discussion

Oncology

3.1.30. EMEA-002585-PIP01-19

Multiple myeloma

Day 30 discussion

Action: For discussion

Oncology

3.1.31. Bintrafusp alfa - Orphan - EMEA-002586-PIP01-19

Merck Europe B.V.; Treatment of all conditions included in the category of malignant neoplasms (except central nervous system tumours, haematopoietic and lymphoid tissue neoplasms) / Treatment of paediatric patients from 3 to <18 years of age with solid malignant tumors

Day 30 discussion

Action: For discussion

Oncology

3.1.32. Emixustat - EMEA-002581-PIP01-19

Stargardt disease

Day 30 discussion

Action: For discussion

Ophthalmology

3.1.33. Timolol / bimatoprost - EMEA-002583-PIP01-19

Ocular hypertension / Primary open-angle glaucoma

Day 30 discussion

Action: For discussion

Ophthalmology

3.1.34. Timrepigene emparvovec - Orphan - EMEA-002430-PIP01-18

Nightstar Europa Limited; Treatment of choroideremia

Day 30 discussion

Action: For discussion

Ophthalmology

3.1.35. EMEA-002559-PIP02-19

Myasthenia gravis

Day 30 discussion

Action: For discussion

Other

3.1.36. Emiplacel - EMEA-002539-PIP02-19

Treatment of muscle injury

Day 30 discussion

Action: For discussion

Other

3.1.37. Adeno-associated viral vector serotype 8 containing the human MTM1 gene - Orphan - EMEA-002571-PIP01-19

Audentes Therapeutics, Inc.; X-linked myotubular myopathy (XLMTM)

Day 30 discussion

Action: For discussion

Other

3.1.38. 2-[[2-ethyl-6-[4-[2-(3-hydroxyazetidin-1-yl)-2-oxoethyl]piperazin-1-yl]-8-methylimidazo[1,2-a]pyridin-3-yl](methyl)amino]-4-(4-fluorophenyl)-1,3-thiazole-5-carbonitrile - Orphan - EMEA-002333-PIP02-19

Galapagos NV; Treatment of idiopathic pulmonary fibrosis, Treatment of interstitial lung disease with fibrosis in children

Day 30 discussion

Action: For discussion

Pneumology - Allergology

3.1.39. EMEA-002612-PIP01-19

Prevention of pulmonary dysfunction / Prevention of cardiopulmonary bypass (CPB) induced postoperative pulmonary dysfunction (PPD)

Day 30 discussion

Action: For discussion

Pneumology - Allergology

3.1.40. Benralizumab - EMEA-001214-PIP03-19

Treatment of vasculitides / Treatment of eosinophilic granulomatosis with polyangiitis (EGPA)

Day 30 discussion

Action: For discussion

Pneumology - Allergology

3.1.41. EMEA-002121-PIP03-19

Treatment of insomnia / Treatment of insomnia in children with comorbid neurodevelopmental and psychiatric disorders

Day 30 discussion

Action: For discussion

Psychiatry

3.1.42. Dasotraline hydrochloride - EMEA-002590-PIP01-19

Binge eating disorder / Moderate to severe binge eating disorder

Day 30 discussion

Action: For discussion

Psychiatry

3.1.43. Ecopipam hydrochloride - EMEA-002564-PIP01-19

Tourette syndrome

Day 30 discussion

Action: For discussion

Psychiatry

3.1.44. EMEA-002589-PIP01-19

Schizophrenia / Treatment of schizophrenia

Day 30 discussion

Action: For discussion

Psychiatry

3.2. Discussions on Compliance Check

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

3.2.1. Terbinafine hydrochloride - EMEA-C-001259-PIP02-13-M02

Polichem, S.A; Treatment of onychomycosis

Day 30 discussion

Action: For discussion

Dermatology

3.2.2. Perampanel - EMEA-C6-000467-PIP01-08-M11

Eisai Europe Ltd; Treatment of treatment-resistant epilepsies

Day 30 discussion

Action: For discussion

Neurology

3.3. Discussions on Modification of an Agreed Paediatric Investigation Plan

No items

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 17 September 2019 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.

5.1. New Scientific Advice

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

5.2. Ongoing Scientific Advice

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

5.3. Final Scientific Advice (Reports and Scientific Advice letters)

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. EMEA-10-2019

All classes of medicinal products for treatment of Alzheimer's disease /

Treatment of Alzheimer's disease

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

No items

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

Update on PDCO member(s)/alternate(s) mandate status

9.1.1. PDCO Chairperson - election

Action: For adoption

9.1.2. August written procedures: process reminder and timelines

Action: For adoption

9.2. Coordination with EMA Scientific Committees or CMDh-v

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

Action: For information

9.3. 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

9.4. Cooperation within the EU regulatory network

9.4.1. European Network of Paediatric Research (Enpr) - European Medicines Agency (EMA): introduction to the European Patient Forum (Youth Group)

Action: For information

9.5. Cooperation with International Regulators

No items

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

9.6.1. Strategic Review and Learning Meeting (SRLM): Selection of therapeutic area/condition overview for November meeting

PDCO: Dirk Mentzer (Chair)

Action: For information

9.6.2. Conect4Children multistakeholder meetings

Action: For information

9.6.3. Unmet medical needs: summary of Malta SRLM presentation and discussion, agreement on the way forward

PDCO members: Karl-Heinz Huemer, Eleni Katsomiti

Action: For information

9.6.4. Strategic Review and Learning Meeting (SRLM) under the Finnish Presidency to be held in Helsinki on 20-22 November 2019

PDCO members: Pia Annunen, Ann-Marie Tötterman

Action: For information

9.7. PDCO work plan

No items

9.8. Planning and reporting

No items

10. Any other business

No items

11. Breakout sessions

11.1.1. Paediatric oncology

Action: For discussion on Thursday, 12:30 – 13:30, room 0-D

11.1.2. Neonatology

Action: For discussion on Thursday, 12:30– 13:30, room 0-F

11.1.3. Inventory

Action: For discussion on Thursday, 12:30 – 13:30, room 1-A

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/