25 February 2020
EMA/PDCO/68068/2020
Inspections, Human Medicines Pharmacovigilance and Committees Division

Paediatric Committee (PDCO)

Draft agenda for the meeting on 25-28 February 2020

Chair: Koenraad Norga – Vice-Chair: Sabine Scherer

25 February 2020, 14:00- 19:00, room 2C

26 February 2020, 08:30- 19:00, room 2C

27 February 2020, 08:30- 19:00, room 2C

28 February 2020, 08:30- 13:00, room 2C

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 25th -28th February 2020. See February 2020 PDCO minutes (to be published post March 2020 PDCO meeting).

1.2. Adoption of agenda

PDCO agenda for 25-28 February 2020

1.3. Adoption of the minutes

PDCO minutes for 28–31 January 2020

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. Lenadogene nolparvovec - Orphan - EMEA-001992-PIP02-16

GenSight-Biologics; Leber Hereditary optic neuropathy (LHON)

Day 120 opinion

Action: For adoption

Ophthalmology

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

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

Day 120 opinion

Action: For adoption

Dermatology

2.1.3. Lebrikizumab - EMEA-002536-PIP01-18

Treatment of atopic dermatitis

Day 120 opinion

Action: For adoption

Dermatology

2.1.4. EMEA-002582-PIP01-19

Treatment of chronic spontaneous urticaria

Day 120 opinion

Action: For adoption

Dermatology

2.1.5. Ladarixin - EMEA-002642-PIP01-19

Treatment of type 1 diabetes / Treatment of new-onset type 1 diabetes mellitus

Day 120 opinion

Action: For adoption

Endocrinology-Gynaecology-Fertility-Metabolism

2.1.6. Alpha1-proteinase inhibitor (human) - EMEA-001312-PIP02-19

Prevention of acute graft-versus-host disease (GVHD)

Day 120 opinion

Action: For adoption

Immunology-Rheumatology-Transplantation

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

Treatment of infections due to aerobic Gram-negative bacteria

Day 120 opinion

Action: For adoption

Infectious Diseases

2.1.8. 5-[[4-[2-[5-(1-Hydroxyethyl)-2-pyridinyl]ethoxy]phenyl]methyl]-2,4-thiazolidinedione hydrochloride - Orphan - EMEA-002106-PIP01-16

Minoryx Therapeutics S.L.; Adrenoleukodystrophy (ALD) / Treatment of adrenomyeloneuropathy (AMN)

Day 120 opinion

Action: For adoption

Neurology

2.1.9. Cenobamate - EMEA-002563-PIP02-19

Treatment of epilepsy

Day 120 opinion

Action: For adoption

Neurology

2.1.10. 1-[4-bromo-5-[1-ethyl-7-(methylamino)-2-oxo-1,2-dihydro-1,6-naphthyridin-3-yl]-2-fluorophenyl]-3-phenylurea - Orphan - EMEA-002526-PIP02-19

Deciphera Pharmaceuticals LLC; Treatment of gastrointestinal stromal tumours

Day 120 opinion

Action: For adoption

Oncology

2.1.11. 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 non-CNS solid malignant tumours

Day 120 opinion

Action: For adoption

Oncology

2.1.12. Pracinostat - Orphan - EMEA-002567-PIP01-19

Helsinn Birex Pharmaceuticals limited; Acute myeloid leukemia / ICD10 code C92.0

Day 120 opinion

Action: For adoption

Oncology

2.1.13. Immunoglobulin G4 - EMEA-002290-PIP01-17

Treatment of all conditions included in the category of malignant neoplasms (except central nervous system tumours, haematopoietic and lymphoid tissue neoplasms) / In combination with nivolumab for the treatment of malignant solid tumours in paediatric patients from 6

months to less than 18 years old

Day 120 opinion

Action: For adoption

Oncology

2.1.14. Temozolomide - EMEA-002634-PIP01-19

Treatment of malignant glioma / Children from the age of three years and adolescents patients with malignant glioma, such as glioblastoma multiforme or anaplastic astrocytoma, showing recurrence or progression after standard therapy, who have difficulty swallowing

Day 120 opinion

Action: For adoption

Oncology

2.1.15. 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 120 opinion

Action: For adoption

Pneumology - Allergology

2.1.16. EMEA-002121-PIP03-19

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

Day 120 opinion

Action: For adoption

Psychiatry

2.1.17. EMEA-002641-PIP01-19

Prevention of pneumococcal disease caused by S. *pneumoniae* / For the active immunisation for the prevention of invasive pneumococcal diseases (IPD) caused by S. *pneumoniae* in infants, children and adolescents from 6 weeks to < 18 years of age

Day 120 opinion

Action: For adoption

Vaccines

2.1.18. Pneumococcal Polyssacharide Serotype 33F conjugated to CRM197 carrier protein and adsorbed on aluminium Phosphate / Pneumococcal Polyssacharide Serotype 23F conjugated to CRM197 carrier protein and adsorbed on aluminium Phosphate / Pneumococcal Polyssacharide Serotype 22F conjugated to CRM197 carrier protein and adsorbed on aluminium Phosphate / Pneumococcal Polyssacharide Serotype 19F conjugated to CRM197 carrier protein and adsorbed on aluminium Phosphate / Pneumococcal Polyssacharide Serotype 19A conjugated to CRM197 carrier protein and adsorbed on aluminium Phosphate / Pneumococcal Polyssacharide Serotype 18C conjugated to CRM197 carrier protein and adsorbed on aluminium Phosphate / Pneumococcal Polyssacharide Serotype 15B conjugated to CRM197 carrier protein and adsorbed on aluminium Phosphate / Pneumococcal Polyssacharide Serotype 14 conjugated to CRM197 carrier protein and adsorbed on aluminium Phosphate / Pneumococcal Polyssacharide Serotype 12F conjugated to CRM197 carrier protein and adsorbed on aluminium Phosphate / Pneumococcal Polyssacharide Serotype 11A conjugated to CRM197 carrier protein and adsorbed on aluminium Phosphate / Pneumococcal Polyssacharide Serotype 10A conjugated to CRM197 carrier protein and adsorbed on aluminium Phosphate / Pneumococcal Polyssacharide Serotype 9V conjugated to CRM197 carrier protein and adsorbed on aluminium Phosphate / Pneumococcal Polyssacharide Serotype 8 conjugated to CRM197 carrier protein and adsorbed on aluminium Phosphate / Pneumococcal Polyssacharide Serotype 7F conjugated to CRM197 carrier protein and adsorbed on aluminium Phosphate / Pneumococcal Polyssacharide Serotype 6B conjugated to CRM197 carrier protein and adsorbed on aluminium Phosphate / Pneumococcal Polyssacharide Serotype 6A conjugated to CRM197 carrier protein and adsorbed on aluminium Phosphate / Pneumococcal Polyssacharide Serotype 5 conjugated to CRM197 carrier protein and adsorbed on aluminium Phosphate / Pneumococcal Polyssacharide Serotype 4 conjugated to CRM197 carrier protein and adsorbed on aluminium Phosphate / Pneumococcal Polyssacharide Serotype 3 conjugated to CRM197 carrier protein and adsorbed on aluminium Phosphate / Pneumococcal Polyssacharide Serotype 1 conjugated to CRM197 carrier protein and adsorbed on aluminium Phosphate - EMEA-002330-PIP01-18

Disease caused by *Streptococcus pneumoniae*

Day 120 opinion

Action: For adoption

Vaccines

2.1.19. Urea / glycerol - EMEA-002511-PIP02-19

Treatment of dry skin

Day 60 opinion

Action: For adoption

Dermatology

2.1.20. Natalizumab - EMEA-001095-PIP03-19

Treatment of multiple sclerosis (MS) / Treatment of relapsing-remitting multiple sclerosis

Day 60 opinion

Action: For adoption

Neurology

2.1.21. EMEA-002619-PIP01-19

Treatment of renal tumours

Day 60 opinion

Action: For adoption

Oncology

2.1.22. EMEA-002714-PIP01-19

Treatment of mature B-cell neoplasms

Day 60 opinion

Action: For adoption

Oncology

2.1.23. Lazertinib - EMEA-002725-PIP01-19

Treatment of lung carcinoma (small cell and non-small cell carcinoma)

Day 60 opinion

Action: For adoption

Oncology

2.1.24. Lanadelumab - Orphan - EMEA-001864-PIP02-19

Shire Pharmaceuticals Ireland Limited (a Takeda company); Prevention of acquired angioedema attacks (AAE) / Prevention of attacks of acquired angioedema (AAE) due to C1-INH deficiency

Day 60 opinion

Action: For adoption

Other

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

Apellis Ireland Limited; Paroxysmal nocturnal haemoglobinuria

Day 120 Opinion

Action: For discussion

Haematology-Hemostaseology

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. Lubiprostone - EMEA-C-000245-PIP01-08-M06

Sucampo AG; Chronic idiopathic constipation

Day 60 opinion

Action: For adoption

Gastroenterology-Hepatology

2.2.2. Avatrombopag (maleate) - EMEA-C1-001136-PIP01-11-M01

Dova Pharmaceuticals Ireland Ltd.; Treatment of idiopathic thrombocytopenic purpura

Day 60 letter

Action: For adoption

Haematology-Hemostaseology

2.3. Opinions on Modification of an Agreed Paediatric Investigation Plan

2.3.1. Tralokinumab - EMEA-001900-PIP02-17-M03

LEO Pharma A/S; Treatment of atopic dermatitis

Day 60 opinion

Action: For adoption

Dermatology

2.3.2. Mirikizumab - EMEA-002208-PIP01-17-M01

Eli Lilly and Company; Treatment of psoriasis / Treatment of Crohn’s disease / Treatment of ulcerative colitis / Treatment of moderate to severely active ulcerative colitis / Treatment of moderate-to-severe plaque psoriasis / Treatment of moderate to severely active Crohn’s disease

Day 60 opinion

Action: For adoption

Dermatology / Gastroenterology-Hepatology

2.3.3. Semaglutide - EMEA-001441-PIP01-13-M03

Novo Nordisk A/S; Type 2 diabetes mellitus / Treatment of diabetes mellitus type 2

Day 60 opinion

Action: For adoption

Endocrinology-Gynaecology-Fertility-Metabolism

2.3.4. Elafibranor - EMEA-001857-PIP01-15-M01

GENFIT SA; Non-alcoholic fatty liver disease (NAFLD) including non-alcoholic steatohepatitis (NASH)

Day 60 opinion

Action: For adoption

Gastroenterology-Hepatology

2.3.5. Etrolizumab - EMEA-001434-PIP01-13-M03

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

2.3.6. Linaclotide - EMEA-000927-PIP01-10-M05

Allergan Pharmaceuticals International Limited; Functional constipation in children

Day 60 opinion

Action: For adoption

Gastroenterology-Hepatology

2.3.7. Boceprevir - EMEA-000583-PIP01-09-M08

Merck Sharp & Dohme (Europe), Inc; Treatment of chronic hepatitis C

Day 60 opinion

Action: For adoption

Infectious Diseases

2.3.8. Tenofovir disoproxil (as fumarate) - EMEA-000533-PIP01-08-M08

Gilead Sciences International Limited; Treatment of chronic viral hepatitis B / For treatment of chronic hepatitis B in paediatric patients from 2 years of age with compensated liver disease

Day 60 opinion

Action: For adoption

Infectious Diseases

2.3.9. Galcanezumab - EMEA-001860-PIP03-16-M04

Eli Lilly and Company Limited; Prevention of migraine headaches

Day 60 opinion

Action: For adoption

Neurology

2.3.10. Lasmiditan - EMEA-002166-PIP01-17-M03

Eli Lilly and Company Limited; Migraine with and without aura

Day 60 opinion

Action: For adoption

Neurology

2.3.11. Autologous peripheral blood T cells CD4 and CD8 selected and CD3 and CD28 activated transduced with retroviral vector expressing anti CD19 CD28/CD3-zeta chimeric antigen receptor and cultured - Orphan - EMEA-001862-PIP01-15-M02

Kite Pharma EU B.V.; Treatment of acute lymphoblastic leukaemia / Treatment of relapsed or refractory B-precursor acute lymphoblastic leukaemia (r/r ALL)

Day 60 opinion

Action: For adoption

Oncology

2.3.12. Axicabtagene ciloleucel - Orphan - EMEA-002010-PIP01-16-M02

Kite Pharma EU B.V.; Treatment of mature B-cell neoplasms / Treatment of children less than 18 years of age and weighing at least 6 kg with relapsed or refractory aggressive B-cell non-Hodgkin lymphoma (NHL) who are ineligible for autologous stem cell transplant

Day 60 opinion

Action: For adoption

Oncology

2.3.13. Blinatumomab - Orphan - EMEA-000574-PIP02-12-M03

Amgen Europe B.V.; Treatment of acute lymphoblastic leukaemia / Treatment of children previously untreated with high-risk first relapse of B-precursor acute lymphoblastic

leukaemia

Day 60 opinion

Action: For adoption

Oncology

2.3.14. Copanlisib - Orphan - EMEA-001757-PIP02-15-M01

Bayer AG; Treatment of all conditions included in the category of malignant neoplasms (except haematopoietic and lymphoid tissue) / Treatment of children with a relapsed or refractory neuroblastoma, Ewing sarcoma, osteosarcoma or rhabdomyosarcoma including at first relapse, in combination with chemotherapy

Day 60 opinion

Action: For adoption

Oncology

2.3.15. Clostridium botulinum neurotoxin type A (150 kD), free from complexing proteins - EMEA-001039-PIP02-12-M04

Merz Pharmaceuticals GmbH; Treatment of sialorrhea / Treatment of chronic troublesome sialorrhea associated with neurological conditions (e.g. cerebral palsy, traumatic brain injury) and/or intellectual disability in children and adolescents aged 2 – 17 years

Day 60 opinion

Action: For adoption

Ophthalmology / Neurology

2.3.16. Palovarotene - Orphan - EMEA-001662-PIP01-14-M03

Ipsen Pharma; Treatment of fibrodysplasia ossificans progressiva

Day 60 opinion

Action: For adoption

Other

2.3.17. Ad26.ZEBOV - EMEA-002307-PIP01-17-M01

Janssen Cilag International NV; Prevention of ebola virus disease (EVD) / Prevention of EVD in children aged ≥1 year

Day 60 opinion

Action: For adoption

Vaccines

2.3.18. MVA-BN-Filo - EMEA-002308-PIP01-17-M01

Janssen Cilag International NV; Prevention of ebola virus disease (EVD) / Prevention of EVD in children aged ≥1 year

Day 60 opinion

Action: For adoption

Vaccines

2.3.19. Regorafenib - EMEA-001178-PIP01-11-M05

Bayer AG; 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 discussion

Oncology

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. Ustekinumab - EMEA-C1-000311-PIP04-13-M01

Janssen-Cilag International NV; Treatment of Crohn's Disease

Day 1 letter

Action: For information

Immunology-Rheumatology-Transplantation / Gastroenterology-Hepatology

2.7.2. Ticagrelor - EMEA-C3-000480-PIP01-08-M11

AstraZeneca AB; Prevention of thromboembolic events

Day 30 letter

Action: For information

Cardiovascular Diseases / Haematology-Hemostaseology

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. Livoletide - Orphan - EMEA-002455-PIP01-18

Millendo Therapeutics SAS; Treatment of Prader-Willi syndrome

Day 90 discussion

Action: For discussion

Endocrinology-Gynaecology-Fertility-Metabolism

3.1.2. Edasalonexent [N-(2-((4Z,7Z,10Z,13Z,16Z,19Z)-docosa-4,7,10,13,16,19-hexaenamido)ethyl)-2-hydroxybenzamide] - Orphan - EMEA-001960-PIP02-16

Catabasis Pharmaceuticals Inc.; Duchenne muscular dystrophy / Treatment of Duchenne muscular dystrophy

Day 90 discussion

Action: For discussion

Neurology

3.1.3. Cyclophosphamide - EMEA-002644-PIP01-19

Treatment of all malignant neoplasms

Day 90 discussion

Action: For discussion

Oncology

3.1.4. Imatinib - EMEA-002643-PIP01-19

Treatment of newly diagnosed Philadelphia chromosome positive acute lymphoblastic leukaemia (Ph+ ALL) integrated with chemotherapy / Treatment of chronic myelogenous

leukaemia: Philadelphia chromosome (Ph1) positive with crisis of blast cells / Paediatric patients with newly diagnosed Philadelphia chromosome (bcr-abl) positive (Ph+) chronic myeloid leukaemia (CML) for whom bone marrow transplantation is not considered as the first line of treatment /Paediatric patients with Ph+ CML in chronic phase after failure of interferon-alpha therapy or in accelerated phase or blast crisis / Paediatric patients with newly diagnosed Philadelphia chromosome positive acute lymphoblastic leukaemia (Ph+ ALL) integrated with chemotherapy

Day 90 discussion

Action: For discussion

Oncology

3.1.5. EMEA-002674-PIP01-19

Treatment of acne vulgaris

Day 60 discussion

Action: For discussion

Dermatology

3.1.6. 2-Thiazolamine, 4-(2-chloro-4-methoxy-5-methylphenyl)-N-[(1S)-2-cyclopropyl-1-(3-fluoro-4-methylphenyl)ethyl]-5-methyl-N-2-propyn-1-yl - Orphan - EMEA-002700-PIP01-19

Neurocrine Therapeutics Ltd; Treatment of congenital adrenal hyperplasia

Day 60 discussion

Action: For discussion

Endocrinology-Gynaecology-Fertility-Metabolism

3.1.7. Lerodalcibep - EMEA-002720-PIP01-19

Treatment of elevated cholesterol / Treatment of elevated low-density lipoprotein cholesterol (LDL-C) in children from 6 to less than 18 years of age with heterozygous familial hypercholesterolaemia (HeFH) or with homozygous familial hypercholesterolaemia (HoFH)

Day 60 discussion

Action: For discussion

Endocrinology-Gynaecology-Fertility-Metabolism

3.1.8. Recombinant human acid alpha-glucosidase - Orphan - EMEA-002447-PIP01-18

Amicus Therapeutics Europe Limited; Treatment of glycogen storage disease type II (Pompe's disease)

Day 60 discussion

Action: For discussion

Endocrinology-Gynaecology-Fertility-Metabolism

3.1.9. Venglustat - Orphan - EMEA-001716-PIP04-19

Genzyme Europe B.V.; E.75.0 GM2 gangliosidosis, E.75.1 Other gangliosidosis, GM1, GM3, E.77.1 Defects in glycoprotein degradation, sialidosis / Long term treatment of patients with a confirmed diagnosis of late onset GM2 gangliosidosis / Long term treatment in patients within the same biochemical pathway as GM2 gangliosidosis / Long term treatment in patients with juvenile (subacute) and adolescent (late-onset) GM2 gangliosidosis ages 2 years old and older, males/females

Day 60 discussion

Action: For discussion

Endocrinology-Gynaecology-Fertility-Metabolism / Neurology

3.1.10. Garadacimab - EMEA-002726-PIP01-19

Hereditary angioedema attacks (HAE)

Day 60 discussion

Action: For discussion

Haematology-Hemostaseology

3.1.11. Recombinant adeno-associated viral vector serotype 6 encoding the B-domain-deleted human factor VIII - Orphan - EMEA-002724-PIP01-19

Pfizer Europe MA EEIG; Treatment of haemophilia A (congenital FVIII deficiency)

Day 60 discussion

Action: For discussion

Haematology-Hemostaseology

3.1.12. Alpha1-proteinase inhibitor (human) - EMEA-001312-PIP03-19

Treatment of acute graft-versus-host disease (GVHD)

Day 60 discussion

Action: For discussion

Immunology-Rheumatology-Transplantation

3.1.13. Doravirine / islatravir - EMEA-002707-PIP01-19

Treatment of human immunodeficiency virus-1 (HIV-1) infection / Doravirine/islatravir is indicated alone or in combination with other antiretroviral medicinal products for the

treatment of HIV-1 infection in paediatric patients from 28 days to less than 18 years of age

Day 60 discussion

Action: For discussion

Infectious Diseases

3.1.14. Vonoprazan - EMEA-002703-PIP01-19

Helicobacter Pylori / Reflux oesophagitis / Treatment of erosive reflux oesophagitis and relief of heartburn / Eradication of helicobacter pylori (H. pylori) concurrently given with appropriate antibiotic therapy

Day 60 discussion

Action: For discussion

Infectious Diseases / Gastroenterology-Hepatology

3.1.15. Autologous CD4 and CD8 T-cells that have been transduced with a self-inactivating lentiviral vector expressing an affinity enhanced NY-ESO-1 specific T-Cell Receptor - Orphan - EMEA-002476-PIP02-19

GlaxoSmithKline Trading Services Limited; Soft tissue sarcoma

Day 60 discussion

Action: For discussion

Oncology

3.1.16. Efbemalenograstim alfa - EMEA-002507-PIP02-19

Prevention of chemotherapy-induced neutropenia and febrile neutropenia / Reduction in the duration of neutropenia and the incidence of febrile neutropenia in patients treated with cytotoxic chemotherapy for malignancy (with the exception of chronic myeloid leukaemia and myelodysplastic syndromes)

Day 60 discussion

Action: For discussion

Oncology

3.1.17. Relatlimab / nivolumab - EMEA-002727-PIP01-19

Treatment of melanoma / Relatlimab/nivolumab fixed dose combination for treatment of unresectable or metastatic melanoma in patients from 12 to 18 years

Day 60 discussion

Action: For discussion

Oncology

3.1.18. Romiplostim - EMEA-000653-PIP02-19

Secondary thrombocytopenia / Treatment of chemotherapy-induced thrombocytopenia (CIT) in children <18 years of age with solid tumours

Day 60 discussion

Action: For discussion

Oncology / Haematology-Hemostaseology

3.1.19. 4-{(2S,4S)-4-Ethoxy-1-[(5-methoxy-7-methyl-1H-indol-4-yl)methyl]piperidin-2-yl}benzoic acid hydrochloride(1/1) - Orphan - EMEA-002705-PIP01-19

Novartis Europharm Limited; C3 glomerulopathy

Day 60 discussion

Action: For discussion

Other

3.1.20. EMEA-002705-PIP02-19

IgA nephropathy

Day 60 discussion

Action: For discussion

Other

3.1.21. Alpelisib - EMEA-002016-PIP03-19

PIK3CA related overgrowth spectrum (PROS)

Day 60 discussion

Action: For discussion

Other

3.1.22. Lanadelumab - Orphan - EMEA-001864-PIP03-19

Shire Pharmaceuticals Ireland Limited (a Takeda company); Prevention of attacks of idiopathic non-histaminergic angioedema (INHA)

Day 60 discussion

Action: For discussion

Other

3.1.23. Macitentan - Orphan - EMEA-001032-PIP03-19

Janssen-Cilag International N.V.; Fontan-palliated patients

Day 30 discussion

Action: For discussion

Cardiovascular Diseases

3.1.24. Spesolimab - EMEA-002475-PIP02-19

Prevention of generalized pustular psoriasis (GPP) / Treatment of GPP / Spesolimab is indicated for treatment of patients with acute or chronic GPP and for the prevention of flares

Day 30 discussion

Action: For discussion

Dermatology

3.1.25. Cotadutide - EMEA-002712-PIP01-19

Treatment of non-cirrhotic non-alcoholic steatohepatitis (NASH) or non-alcoholic fatty liver disease (NAFLD) / For the resolution of steatohepatitis with no worsening of fibrosis in obese children and adolescents with non-cirrhotic non-alcoholic steatohepatitis (NASH) and non-alcoholic fatty liver disease (NAFLD)

Day 30 discussion

Action: For discussion

Endocrinology-Gynaecology-Fertility-Metabolism

3.1.26. Fenofibrate / rosuvastatin (calcium) - EMEA-002743-PIP01-19

Cardiovascular risk with mixed dyslipidaemia

Day 30 discussion

Action: For discussion

Endocrinology-Gynaecology-Fertility-Metabolism / Cardiovascular Diseases

3.1.27. DTX401 - Orphan - EMEA-002734-PIP01-19

Ultragenyx Germany GmbH; Treatment of glycogen storage disease type Ia

Day 30 discussion

Action: For discussion

Endocrinology-Gynaecology-Fertility-Metabolism

3.1.28. Benzocaine / hydrocortisone - EMEA-002739-PIP01-19

Grade II hemorrhoids / Grade I hemorrhoids / Local relief of pain, itching, burning and inflammation associated with hemorrhoids

Day 30 discussion

Action: For discussion

Gastroenterology-Hepatology

3.1.29. Etrasimod L-arginine - EMEA-002713-PIP01-19

Treatment of ulcerative colitis / Treatment of moderately or severely active ulcerative colitis

Day 30 discussion

Action: For discussion

Gastroenterology-Hepatology

3.1.30. Autologous CD34+ hematopoietic stem cells with a CRISPR-edited erythroid enhancer region of the BCL11A gene - Orphan - EMEA-002730-PIP01-19

Vertex Pharmaceuticals (Ireland) Limited; Treatment of beta-thalassemia / Treatment of transfusion-dependent beta-thalassemia

Day 30 discussion

Action: For discussion

Haematology-Hemostaseology

3.1.31. Etranacogene dezaparvovec - Orphan - EMEA-002722-PIP01-19

uniQure biopharma B.V.; Treatment of haemophilia B

Day 30 discussion

Action: For discussion

Haematology-Hemostaseology

3.1.32. Plasma kallikrein inhibitor - EMEA-002723-PIP01-19

Treatment of hereditary angioedema

Day 30 discussion

Action: For discussion

Haematology-Hemostaseology

3.1.33. Ibrexafungerp - EMEA-002535-PIP03-19

Vulvovaginal candidiasis

Day 30 discussion

Action: For discussion

Infectious Diseases

3.1.34. Adeno-associated virus serotype rh74 containing a human micro-dystrophin gene - EMEA-002677-PIP01-19

Duchenne muscular dystrophy

Day 30 discussion

Action: For discussion

Neurology

3.1.35. Diroximel - EMEA-002685-PIP02-19

Treatment of multiple sclerosis

Day 30 discussion

Action: For discussion

Neurology

3.1.36. 18-(p-[131I]-iodophenyl)octadecyl phosphocholine - Orphan - EMEA-002745-PIP01-19

Cellectar Biosciences, Inc.; Multiple myeloma / Mature B-cell lymphomas

Day 30 discussion

Action: For discussion

Oncology

3.1.37. Padsevonil - EMEA-002466-PIP02-19

Treatment of fixation off sensitivity (FOS) in patients with epilepsy / Adjunctive treatment of FOS in paediatric patients with epilepsy

Day 30 discussion

Action: For discussion

Neurology

3.1.38. Arfolitixorin - EMEA-002223-PIP01-19

Treatment of colorectal cancer

Day 30 discussion

Action: For discussion

Oncology

3.1.39. Idasanutlin - Orphan - EMEA-001489-PIP02-19

Roche Registration GmbH; Treatment of all conditions included in the category of malignant neoplasms (except nervous system, haematopoietic and lymphoid tissue) / Treatment of children with a solid malignant tumour and which is newly-diagnosed and metastatic, or refractory to first-line treatment

Day 30 discussion

Action: For discussion

Oncology

3.1.40. EMEA-002716-PIP01-19

Treatment of all conditions included in the category of malignant neoplasms (except haematopoietic and lymphoid tissue neoplasms)

Day 30 discussion

Action: For discussion

Oncology

3.1.41. Tiragolumab - EMEA-002721-PIP01-19

Treatment of lung carcinoma (small cell and non-small cell carcinoma)

Day 30 discussion

Action: For discussion

Oncology

3.1.42. 17-mer, 2'-O-methyl modified phosphorothioate RNA oligonucleotide - Orphan - EMEA-002717-PIP01-19

ProQR Therapeutics; Treatment of inherited retinal disorders / Treatment of Leber's congenital amaurosis due to the p.Cys998X mutation (C2991 +1655A>G) in the CEP290 gene

Day 30 discussion

Action: For discussion

Ophthalmology

3.1.43. Difelikefalin - EMEA-002565-PIP02-19

Chronic kidney disease (CKD)-associated pruritus

Day 30 discussion

Action: For discussion

Other

3.1.44. Recifercept - Orphan - EMEA-002715-PIP01-19

Pfizer Europe MA EEIG; Treatment of achondroplasia

Day 30 discussion

Action: For discussion

Other

3.1.45. EMEA-002731-PIP01-19

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. Ambrisentan - EMEA-C2-000434-PIP01-08-M06

Glaxo Group Limited; Treatment of pulmonary arterial hypertension

Day 30 discussion

Action: For discussion

Cardiovascular Diseases

3.2.2. Sitagliptin phosphate - EMEA-C-000470-PIP01-08-M11

Merck Sharp & Dohme (Europe), Inc.; Type 2 diabetes mellitus

Day 30 discussion

Action: For discussion

Endocrinology-Gynaecology-Fertility-Metabolism

3.2.3. Cabozantinib (S)-malate - EMEA-C1-001143-PIP01-11

Ipsen Pharma; Treatment of malignant solid tumours

Day 30 discussion

Action: For discussion

Oncology

3.2.4. Mepolizumab - EMEA-C-000069-PIP04-13-M02

GSK Trading Services Limited; Treatment of vasculitides

Day 30 discussion

Action: For discussion

Pneumology - Allergology

3.3. Discussions on Modification of an Agreed Paediatric Investigation Plan

3.3.1. Crisaborole - EMEA-002065-PIP01-16-M02

Pfizer Europe MA EEIG; Mild to moderate atopic dermatitis

Day 30 discussion

Action: For discussion

Dermatology

3.3.2. Avalglucosidase alfa - Orphan - EMEA-001945-PIP01-16-M02

Genzyme Europe B.V.; ICD-10: E74.0; Glycogen storage disease (Pompe disease) / Long-term use as an enzyme replacement therapy (ERT) for the treatment of patients with a confirmed diagnosis of Pompe disease (acid α-glucosidase deficiency)

Day 30 discussion

Action: For discussion

Endocrinology-Gynaecology-Fertility-Metabolism

3.3.3. Dulaglutide - EMEA-000783-PIP01-09-M05

Eli Lilly and Company; Treatment of type 2 diabetes mellitus

Day 30 discussion

Action: For discussion

Endocrinology-Gynaecology-Fertility-Metabolism

3.3.4. Romosozumab - EMEA-001075-PIP04-15-M02

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

Day 30 discussion

Action: For discussion

Endocrinology-Gynaecology-Fertility-Metabolism

3.3.5. Teplizumab - EMEA-000524-PIP01-08-M02

Provention Bio, Inc.; Recent-onset type I diabetes mellitus

Day 30 discussion

Action: For discussion

Endocrinology-Gynaecology-Fertility-Metabolism

3.3.6. Volanesorsen - Orphan - EMEA-001915-PIP01-15-M02

Akcea Therapeutics; Familial chylomicronemia syndrome

Day 30 discussion

Action: For discussion

Endocrinology-Gynaecology-Fertility-Metabolism

3.3.7. Alicaforsen (as sodium salt) - Orphan - EMEA-002060-PIP02-17-M01

Atlantic Healthcare Europe B.V.; Treatment of gastrointestinal procedural complications / Treatment of active episodes of antibiotic refractory pouchitis

Day 30 discussion

Action: For discussion

Gastroenterology-Hepatology

3.3.8. Tofacitinib - EMEA-000576-PIP03-12-M03

Pfizer Europe MA EEIG; Treatment of ulcerative colitis

Day 30 discussion

Action: For discussion

Gastroenterology-Hepatology

3.3.9. 2-iminobiotin - Orphan - EMEA-001070-PIP01-10-M02

Neurophyxia BV; Perinatal asphyxia / Treatment of perinatal asphyxia

Day 30 discussion

Action: For discussion

Neonatology - Paediatric Intensive Care

3.3.10. Isoflurane - EMEA-002320-PIP01-17-M01

Sedana Medical AB; Sedation of mechanically ventilated patients

Day 30 discussion

Action: For discussion

Neonatology - Paediatric Intensive Care

3.3.11. Dimethyl fumarate - EMEA-000832-PIP01-10-M05

Biogen Idec Ltd.; Multiple Sclerosis / Treatment of relapsing remitting forms of multiple sclerosis

Day 30 discussion

Action: For discussion

Neurology

3.3.12. Pitolisant - Orphan - EMEA-001176-PIP01-11-M04

BIOPROJET PHARMA; Narcolepsy / Narcolepsy with or without cataplexy

Day 30 discussion

Action: For discussion

Neurology

3.3.13. Setmelanotide - Orphan - EMEA-002209-PIP01-17-M01

Rhythm Pharmaceuticals, Inc; Treatment of appetite and general nutrition disorders / Treatment of obesity and/or hyperphagia associated with genetic defects upstream of the MC4 receptor in the leptin-melanocortin pathway

Day 30 discussion

Action: For discussion

Nutrition

3.3.14. Afatinib - EMEA-001596-PIP02-17-M02

Boehringer Ingelheim International GmbH; Treatment of all conditions included in the category of malignant neoplasms (except central nervous system, haematopoietic and lymphoid tissue neoplasms) / Treatment of malignant neoplasms of the central nervous system

Day 30 discussion

Action: For discussion

Oncology

3.3.15. Talimogene laherparepvec - EMEA-001251-PIP01-11-M04

Amgen Europe B.V.; Melanoma / Treatment of adolescent patients with unresectable stage IIIB/C/IVM1a melanoma

Day 30 discussion

Action: For discussion

Oncology

3.3.16. Gilteritinib (as fumarate) - Orphan - EMEA-002064-PIP01-16-M02

Astellas Pharma Europe B.V.; Treatment of acute myeloid leukemia / Treatment of patients from 6 months to less than 18 years of age with relapsed or refractory FLT3/ITD positive acute myeloid leukaemia or newly-diagnosed FLT3/ITD positive acute myeloid leukaemia

Day 30 discussion

Action: For discussion

Oncology / Haematology-Hemostaseology

3.3.17. Ivacaftor - Orphan - EMEA-000335-PIP01-08-M14

Vertex Pharmaceuticals (Ireland) Ltd; Cystic fibrosis (E84 of ICD10) / Treatment of cystic fibrosis

Day 30 discussion

Action: For discussion

Other

3.3.18. ROLAPITANT - EMEA-001768-PIP02-15-M03

Tesaro Bio Netherlands B.V.; Chemotherapy-induced nausea and vomiting (CINV) in subjects receiving highly emetogenic chemotherapy (HEC)

Day 30 discussion

Action: For discussion

Other

3.3.19. Methoxyflurane - EMEA-000334-PIP01-08-M09

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

Day 30 discussion

Action: For discussion

Pain

3.3.20. Dupilumab - EMEA-001501-PIP02-13-M05

sanofi-aventis recherche & développement; Treatment of asthma

Day 30 discussion

Action: For discussion

Pneumology - Allergology

3.3.21. Ivacaftor / tezacaftor - Orphan - EMEA-001640-PIP01-14-M06

Vertex Pharmaceuticals (Europe) Ltd.; Cystic fibrosis / Treatment of cystic fibrosis

Day 30 discussion

Action: For discussion

Pneumology - Allergology

3.3.22. Influenza virus surface antigens - A/turkey/turkey/1/05 (H5N1) - EMEA-000599-PIP01-09-M07

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

Day 30 discussion

Action: For discussion

Vaccines

3.3.23. Pandemic influenza vaccine (H5N1) (surface antigen, inactivated, adjuvanted) - EMEA-001830-PIP01-15-M02

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

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 31 March 2020 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. Estradiol / progesterone - EMEA-21-2019

Theramex Ireland Ltd; All classes of medicinal products for treatment of climacteric

symptoms associated with decreased oestrogen levels, as occurring at menopause/ Hormone replacement therapy for oestrogen deficiency symptoms in postmenopausal women

Action: For adoption

6.1.2. EMEA-22-2019

Roche Registration GmbH; The classes of oestrogen receptor modulator medicinal products for treatment of breast malignant neoplasms / Indicated, in combination with palbociclib, for the treatment of women with oestrogen receptor-positive (ER +) HER2-negative (HER2 -) previously untreated locally advanced or metastatic breast cancer (MBC)

Action: For adoption

6.1.3. EMEA-01-2020

Eisai GmbH; The classes of oestrogen receptor modulator medicinal products for treatment of breast malignant neoplasms / Treatment of locally advanced or metastatic oestrogen receptor-positive, HER2 (human epidermal growth factor receptor 2)-negative breast cancer

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.

No items

9. Organisational, regulatory and methodological matters

9.1. Mandate and organisation of the PDCO

No items

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

9.3.3. Extrapolation - update on the guidance template

Action: For information

9.3.4. Meeting Summary from the Annual Patients and Consumers Working Party (PCWP)/ Healthcare Professionals Working Party (HCPWP) with all eligible organisations - 20 November 2019

Action: For information

9.3.5. Agenda for the Patients and Consumers Working Party (PCWP)/ Healthcare Professionals Working Party (HCPWP) on 03-04 March 2020

Action: For information

9.4. Cooperation within the EU regulatory network

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

PDCO representation in Enpr-EMA's Coordinating 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. Fifth Accelerate Paediatric Strategy Forum for Medicinal Product Development of Epigenetic Modifiers in Children – feedback from the meeting

Action: For information

9.7. PDCO work plan

No items

9.8. Planning and reporting

No items

10. Any other business

10.1.1. UK withdrawal from the EU - update

Action: For discussion

11. Breakout sessions

11.1.1. Paediatric oncology

Action: For discussion on Thursday, 14:00 - 15:00, room 2A

11.1.2. Neonatology

Action: For discussion on Thursday, 14:00 - 15:00, room 2D

11.1.3. Inventory

Action: For discussion on Thursday, 14:00 - 15:00, room 2B

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/