24 March 2020
EMA/PDCO/116804/2020
Inspections, Human Medicines Pharmacovigilance and Committees Division

Paediatric Committee (PDCO)

Agenda for the meeting on 24-27 March 2020

Chair: Koenraad Norga – Vice-Chair: Sabine Scherer

24 March 2020, 14:00- 17:00, 2C / Virtual meeting

25 March 2020, 08:30- 19:00, 2C / Virtual meeting

26 March 2020, 08:30- 19:00, 2C / Virtual meeting

27 March 2020, 08:30- 13:00, 2C / Virtual meeting

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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Table of contents

1. Introductions

1.1. Welcome and declarations of interest of members, alternates and experts

Pre-meeting list of participants and restrictions in relation to declarations of interests applicable to the items of the agenda for the PDCO plenary session to be held 24-27 March 2020. See 24-27 March 2020 PDCO minutes (to be published post 28-30 April PDCO meeting).

1.2. Adoption of agenda

PDCO agenda for 24-27 March 2020

1.3. Adoption of the minutes

PDCO minutes for 25-28 February 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. Livoletide - Orphan - EMEA-002455-PIP01-18

Millendo Therapeutics SAS; Treatment of Prader-Willi syndrome

Day 120 opinion

Action: For adoption

Endocrinology-Gynaecology-Fertility-Metabolism

2.1.2. Cyclophosphamide - EMEA-002644-PIP01-19

Treatment of all malignant neoplasms

Day 120 opinion

Action: For adoption

Oncology

2.1.3. 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 120 opinion

Action: For adoption

Oncology

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

Cardiovascular risk with mixed dyslipidaemia

Day 60 opinion

Action: For adoption

Endocrinology-Gynaecology-Fertility-Metabolism / Cardiovascular Diseases

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

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

Day 60 opinion

Action: For adoption

Gastroenterology-Hepatology

2.1.6. 18-(p-[131I]-iodophenyl)octadecyl phosphocholine - Orphan - EMEA-002745-PIP01-19

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

Day 60 opinion

Action: For adoption

Oncology

2.1.7. Arfolitixorin - EMEA-002223-PIP01-19

Treatment of colorectal cancer

Day 60 opinion

Action: For adoption

Oncology

2.1.8. EMEA-002716-PIP01-19

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

Day 60 opinion

Action: For adoption

Oncology

2.1.9. Tiragolumab - EMEA-002721-PIP01-19

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

Day 60 opinion

Action: For adoption

Oncology

2.1.10. 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 60 opinion

Action: For discussion

Oncology

2.1.11. Spesolimab - EMEA-002475-PIP02-19

Prevention of generalized pustular psoriasis (GPP) / Treatment of GPP

Day 60 opinion

Action: For adoption

Dermatology

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. Lonafarnib - EMEA-C-002516-PIP01-18

EigerBio Europe Limited; Hutchinson-Gilford progeria syndrome (HGPS) / Progeroid laminopathies / For the treatment of Hutchinson-Gilford progeria syndrome (HGPS) and progeroid laminopathies in children at age of 12 months and older

Day 1 opinion

Action: For adoption

2.2.2. Romiplostim - EMEA-C-000653-PIP01-09-M05

Amgen Europe B.V.; Treatment of immune thrombocytopenia (idiopathic thrombocytopenic purpura)

Day 30 opinion

Action: For adoption

Haematology-Hemostaseology

2.3. Opinions on Modification of an Agreed Paediatric Investigation Plan

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

Pfizer Europe MA EEIG; Mild to moderate atopic dermatitis

Day 60 opinion

Action: For adoption

Dermatology

2.3.2. 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 60 opinion

Action: For adoption

Endocrinology-Gynaecology-Fertility-Metabolism

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

Eli Lilly and Company; Treatment of type 2 diabetes mellitus

Day 60 opinion

Action: For adoption

Endocrinology-Gynaecology-Fertility-Metabolism

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

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

Day 60 opinion

Action: For adoption

Endocrinology-Gynaecology-Fertility-Metabolism

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

Akcea Therapeutics; Familial chylomicronemia syndrome

Day 60 opinion

Action: For adoption

Endocrinology-Gynaecology-Fertility-Metabolism

2.3.6. 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 60 opinion

Action: For adoption

Gastroenterology-Hepatology

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

Pfizer Europe MA EEIG; Treatment of ulcerative colitis

Day 60 opinion

Action: For adoption

Gastroenterology-Hepatology

2.3.8. 2-iminobiotin - Orphan - EMEA-001070-PIP01-10-M02

Neurophyxia BV; Perinatal asphyxia / Treatment of perinatal asphyxia

Day 60 opinion

Action: For adoption

Neonatology - Paediatric Intensive Care

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

Sedana Medical AB; Sedation of mechanically ventilated patients

Day 60 opinion

Action: For adoption

Neonatology - Paediatric Intensive Care

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

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

Day 60 opinion

Action: For adoption

Neurology

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

BIOPROJET PHARMA; Narcolepsy / Narcolepsy with or without cataplexy

Day 60 opinion

Action: For adoption

Neurology

2.3.12. 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 60 opinion

Action: For adoption

Nutrition

2.3.13. 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 60 opinion

Action: For adoption

Oncology

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

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

Day 60 opinion

Action: For adoption

Oncology

2.3.15. 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 60 opinion

Action: For adoption

Oncology / Haematology-Hemostaseology

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

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

Day 60 opinion

Action: For adoption

Other

2.3.17. 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 60 opinion

Action: For adoption

Other

2.3.18. 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 60 opinion

Action: For adoption

Pain

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

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

Day 60 opinion

Action: For adoption

Pneumology - Allergology

2.3.20. 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 60 opinion

Action: For adoption

Vaccines

2.3.21. 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 60 opinion

Action: For adoption

Vaccines

2.4. Opinions on Re-examinations

2.4.1. Macimorelin - EMEA-001988-PIP01-16-M01

Aeterna Zentaris GmbH; Growth hormone deficiency / Diagnosis of growth hormone deficiency

Day 30 opinion

Action: For adoption

Endocrinology-Gynaecology-Fertility-Metabolism / Diagnostic

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. 6-cyclopropaneamido-4-{[2-methoxy-3-(1-methyl-1H-1,2,4 triazol-3-yl)phenyl]amino}-N-(2H3)methylpyridazine-3-carboxamide (BMS-986165) - EMEA-C1-002350-PIP01-18

Bristol-Myers Squibb International Corporation; Treatment of psoriasis

Day 1 letter

Action: For information

Dermatology

2.7.2. Upadacitinib - EMEA-C2-001741-PIP01-14-M02

AbbVie Ltd; Treatment of chronic idiopathic arthritis (including rheumathoid arthritis, psoriatic arthritis, spondyloarthritis and juvenile idiopathic arthritis)

Day 1 letter

Action: For information

Immunology-Rheumatology-Transplantation

2.7.3. Linagliptin - EMEA-C1-000498-PIP01-08-M08

Boehringer Ingelheim International GmbH; Treatment of type 2 diabetes mellitus

Day 1 letter

Action: For information

Endocrinology-Gynaecology-Fertility-Metabolism

2.7.4. Lumasiran sodium - EMEA-C1-002079-PIP01-16-M01

Alnylam UK Limited; Treatment hyperoxaluria

Day 1 letter

Action: For information

Uro-nephrology

2.8. Revision of PDCO Opinions

No items

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. Bis-choline tetrathiomolybdate - Orphan - EMEA-002232-PIP02-19

Alexion Europe S.A.S.; Wilson disease

Day 90 discussion

Action: For discussion

Endocrinology-Gynaecology-Fertility-Metabolism

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

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

Day 90 discussion

Action: For discussion

Haematology-Hemostaseology

3.1.3. Fidanacogene elaparvovec - Orphan - EMEA-002362-PIP02-19

Pfizer Europe MA EEIG; Treatment of congenital factor IX deficiency (haemophilia B) / Prophylaxis of haemophilia B (congenital factor IX deficiency)

Day 90 discussion

Action: For discussion

Haematology-Hemostaseology

3.1.4. Gepotidacin - EMEA-002443-PIP01-18

Treatment of uncomplicated urinary tract infection (uUTI) / Treatment of uncomplicated urinary tract infection (acute cystitis) in children aged >2 years to <18 years

Day 90 discussion

Action: For discussion

Infectious Diseases

3.1.5. Gepotidacin - EMEA-002443-PIP02-18

Treatment of uncomplicated urogenital gonorrhea (GC) / Treatment of uncomplicated urogenital gonorrhea in children ≥ 12 to <18 years

Day 90 discussion

Action: For discussion

Infectious Diseases

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

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

Day 90 discussion

Action: For discussion

Neurology

3.1.7. Autologous inactivated glioma cells - Orphan - EMEA-002663-PIP01-19

ERC Belgium; Recurrent high grade glioma / Treatment of recurrent high grade glioma

Day 90 discussion

Action: For discussion

Oncology

3.1.8. Autologous inactivated glioma cells - Orphan - EMEA-002662-PIP01-19

ERC Belgium; Recurrent high grade glioma / Treatment of recurrent high grade glioma

Day 90 discussion

Action: For discussion

Oncology

3.1.9. Autologous inactivated glioma cells - Orphan - EMEA-002664-PIP01-19

ERC Belgium; Recurrent high grade glioma / Treatment of recurrent high grade glioma

Day 90 discussion

Action: For discussion

Oncology

3.1.10. Autologous inactivated glioma cells - Orphan - EMEA-002661-PIP01-19

ERC Belgium; Recurrent high grade glioma / Treatment of recurrent high grade glioma

Day 90 discussion

Action: For discussion

Oncology

3.1.11. Lenvatinib - EMEA-001119-PIP03-19

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

Day 90 discussion

Action: For discussion

Oncology

3.1.12. Trilaciclib - EMEA-002534-PIP02-19

Prevention of chemotherapy induced myelosuppression

Day 90 discussion

Action: For discussion

Oncology

3.1.13. 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 90 discussion

Action: For discussion

Ophthalmology

3.1.14. Atropine - EMEA-002545-PIP01-19

Myopia

Day 90 discussion

Action: For discussion

Ophthalmology

3.1.15. Sodium alginate oligosaccharide - Orphan - EMEA-002321-PIP01-17

AlgiPharma AS; Symptomatic treatment of cystic fibrosis

Day 90 discussion

Action: For discussion

Pneumology - Allergology

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

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

Day 60 discussion

Action: For discussion

Cardiovascular Diseases

3.1.17. 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 60 discussion

Action: For discussion

Endocrinology-Gynaecology-Fertility-Metabolism

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

Ultragenyx Germany GmbH; Treatment of glycogen storage disease type IA

Day 60 discussion

Action: For discussion

Endocrinology-Gynaecology-Fertility-Metabolism

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

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

Day 60 discussion

Action: For discussion

Gastroenterology-Hepatology

3.1.20. 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 60 discussion

Action: For discussion

Haematology-Hemostaseology

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

uniQure biopharma B.V.; Treatment of haemophilia B

Day 60 discussion

Action: For discussion

Haematology-Hemostaseology

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

Treatment of hereditary angioedema

Day 60 discussion

Action: For discussion

Haematology-Hemostaseology

3.1.23. Ibrexafungerp - EMEA-002535-PIP03-19

Vulvovaginal candidiasis

Day 60 discussion

Action: For discussion

Infectious Diseases

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

Duchenne muscular dystrophy

Day 60 discussion

Action: For discussion

Neurology

3.1.25. Diroximel - EMEA-002685-PIP02-19

Treatment of multiple sclerosis

Day 60 discussion

Action: For discussion

Neurology

3.1.26. 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 60 discussion

Action: For discussion

Neurology

3.1.27. 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 60 discussion

Action: For discussion

Ophthalmology

3.1.28. Difelikefalin - EMEA-002565-PIP02-19

Chronic kidney disease (CKD)-associated pruritus

Day 60 discussion

Action: For discussion

Other

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

Pfizer Europe MA EEIG; Treatment of achondroplasia

Day 60 discussion

Action: For discussion

Other

3.1.30. EMEA-002731-PIP01-19

Treatment of schizophrenia

Day 60 discussion

Action: For discussion

Psychiatry

3.1.31. Chloroprocaine - EMEA-000639-PIP06-20

Ocular surface anaesthesia

Day 30 discussion

Action: For discussion

Anaesthesiology

3.1.32. Ezetimibe / atorvastatin - EMEA-002649-PIP02-20

Prevention of cardiovascular events / Atorvastatin/ezetimibe is indicated as substitution therapy to reduce the risk of cardiovascular events in patients with coronary heart disease (CHD) and a history of acute coronary syndrome (ACS), for adults receiving atorvastatin and ezetimibe concurrently at the same dose level

Day 30 discussion

Action: For discussion

Cardiovascular Diseases

3.1.33. Bilastine - EMEA-000347-PIP05-20

Acute urticaria / Short-term treatment as single therapy or in severe cases as additional therapy option of histamine-mediated type I hypersensitivity reactions, such as acute urticaria, when immediate action is required or parenteral formulation is preferred

Day 30 discussion

Action: For discussion

Dermatology / Pneumology - Allergology / Oto-rhino-laryngology

3.1.34. Semaglutide - EMEA-001441-PIP04-20

Treatment of non-alcoholic fatty liver disease (NAFLD)

Day 30 discussion

Action: For discussion

Gastroenterology-Hepatology

3.1.35. Autologous CD34+ hematopoietic stem cells with a CRISPR-edited erythroid enhancer region of the BCL11A gene - Orphan - EMEA-002730-PIP02-19

Vertex Pharmaceuticals (Ireland) Limited; Treatment of sickle cell disease

Day 30 discussion

Action: For discussion

Haematology-Hemostaseology

3.1.36. N-[(2S)-5-{[(1R, 2S)-2-(4-fluorophenyl)cyclopropyl] amino}-1-(4-methylpiperazin-1-yl)-1-oxopentan-2-yl]-4-(1H-1,2,3-triazol-1-yl)benzamide, bis-tosylate salt - Orphan - EMEA-002752-PIP01-19

Imago Biosciences BV; Treatment of myeloproliferative neoplasms

Day 30 discussion

Action: For discussion

Haematology-Hemostaseology

3.1.37. Bimezikumab - EMEA-002189-PIP03-19

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

Day 30 discussion

Action: For discussion

Immunology-Rheumatology-Transplantation

3.1.38. EMEA-002742-PIP01-19

Treatment of parainfluenza infection

Day 30 discussion

Action: For discussion

Infectious Diseases

3.1.39. Adeno-associated virus serotype 2 (AAV2) encoding human aromatic L-amino acid decarboxylase (hAADC) - EMEA-002753-PIP01-19

Parkinson's disease

Day 30 discussion

Action: For discussion

Neurology

3.1.40. 1-[({4-[(4-fluoro-2-methyl-1H-indol-5-yl)oxy]-6-methoxyquinolin-7-yl}oxy)methyl]cyclopropan-1-amine - Orphan - EMEA-002486-PIP03-20

Advenchen Laboratories, LLC.; Treatment of soft tissue sarcomas / Treatment of alveolar soft part sarcoma / Treatment of synovial sarcoma

Day 30 discussion

Action: For discussion

Oncology

3.1.41. Monalizumab - EMEA-002751-PIP01-19

Head and neck epithelial malignant neoplasms

Day 30 discussion

Action: For discussion

Oncology

3.1.42. Ribociclib - EMEA-002765-PIP01-19

Treatment of breast cancer

Day 30 discussion

Action: For discussion

Oncology

3.1.43. Atropine - EMEA-002744-PIP01-19

Myopia / Treatment to slow myopia progression

Day 30 discussion

Action: For discussion

Ophthalmology

3.1.44. EMEA-002748-PIP01-20

Secondary lymphedema associated with the treatment of breast cancer

Day 30 discussion

Action: For discussion

Other

3.1.45. Rituximab / CD3+CD4+CD25+CD127-FoxP3+ regulatory T cells - EMEA-002737-PIP01-19

Treatment of type 1 diabetes mellitus (T1DM)

Day 30 discussion

Action: For discussion

Other / Endocrinology-Gynaecology-Fertility-Metabolism

3.1.46. Betahistine - EMEA-002652-PIP01-19

Acute peripheral vertigo

Day 30 discussion

Action: For discussion

Oto-rhino-laryngology / Neurology

3.1.47. Dexmedetomidine - EMEA-002758-PIP01-19

Treatment of acute agitation in bipolar disorder / Treatment of acute agitation in schizophrenia

Day 30 discussion

Action: For discussion

Psychiatry

3.1.48. Allopurinol / verinurad - EMEA-002754-PIP01-19

Chronic kidney disease / Treatment of chronic kidney disease in children and adolescents (6 to <18 years old) with hyperuricaemia and albuminuria

Day 30 discussion

Action: For discussion

Uro-nephrology

3.1.49. EMEA-002630-PIP01-19

Chikungunya virus infection

Day 30 discussion

Action: For discussion

Vaccines / Infectious Diseases

3.2. Discussions on Compliance Check

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

3.2.1. Zoledronic acid - EMEA-C-000057-PIP01-07-M07

Novartis Europharm Limited; Treatment of osteoporosis

Day 30 discussion

Action: For discussion

Endocrinology-Gynaecology-Fertility-Metabolism

3.2.2. Adalimumab - EMEA-C-000366-PIP02-09-M06

AbbVie Limited; Treatment of ulcerative colitis

Day 30 discussion

Action: For discussion

Gastroenterology-Hepatology

3.2.3. Palonosetron / fosnetupitant - EMEA-C1-001198-PIP03-17-M03

Helsinn Birex Pharmaceuticals Limited; Prevention of chemotherapy-induced nausea and vomiting

Day 30 discussion

Action: For discussion

Gastroenterology-Hepatology

3.2.4. (3R,4R)-4-methyl-3-(methyl-7H-pyrrolo[2,3-d]pyrimidin-4-ylamino)-β-oxo-1-piperidinepropanenitrile, 2-hydroxy-1,2,3-propanetricarboxylate (1:1)(Company code CP-690,550-10) - EMEA-C4-000576-PIP01-09-M10

Pfizer Europe MA EEIG; Treatment of chronic idiopathic arthritis (including rheumatoid arthritis, psoriatic arthritis, ankylosing spondylitis and juvenile idiopathic arthritis)

Day 30 discussion

Action: For discussion

Immunology-Rheumatology-Transplantation

3.2.5. Cilastatin sodium / relebactam / imipenem monohydrate - EMEA-C2-001809-PIP01-15-M01

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

Day 30 discussion

Action: For discussion

Infectious Diseases

3.2.6. Erenumab - EMEA-C2-001664-PIP02-15-M03

Novartis Europharm Limited; Prevention of migraine headaches

Day 30 discussion

Action: For discussion

Neurology

3.2.7. Inebilizumab - EMEA-C3-001911-PIP01-15-M02

Viela Bio; Treatment of neuromyelitis optica spectrum disorders

Day 30 discussion

Action: For discussion

Neurology

3.2.8. Olopatadine (hydrochloride) / mometasone (furoate monohydrate) - EMEA-C-002514-PIP01-18

Glenmark Pharmaceuticals Europe Ltd.; Treatment of allergic rhinitis / rhino-conjunctivitis

Day 30 discussion

Action: For discussion

Oto-rhino-laryngology

3.3. Discussions on Modification of an Agreed Paediatric Investigation Plan

3.3.1. Apixaban - EMEA-000183-PIP01-08-M08

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

Day 30 discussion

Action: For discussion

Cardiovascular Diseases

3.3.2. Apixaban - EMEA-000183-PIP02-12-M03

Bristol-Myers Squibb / Pfizer EEIG; Treatment of venous thromboembolism

Day 30 discussion

Action: For discussion

Cardiovascular Diseases

3.3.3. Etripamil - EMEA-002303-PIP01-17-M01

Milestone Pharmaceuticals, Inc.; Treatment of supraventricular tachychardia / Treatment of acute paroxysmal supraventricular tachycardia (PSVT)

Day 30 discussion

Action: For discussion

Cardiovascular Diseases

3.3.4. Ticagrelor - EMEA-000480-PIP01-08-M13

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 30 discussion

Action: For discussion

Cardiovascular Diseases / Haematology-Hemostaseology

3.3.5. Ligelizumab - EMEA-001811-PIP02-15-M03

Novartis Europharm Limited; Treatment of chronic spontaneous urticaria

Day 30 discussion

Action: For discussion

Dermatology

3.3.6. Lucerastat - Orphan - EMEA-002095-PIP01-16-M01

Idorsia Pharmaceuticals Deutschland GmbH; Treatment of Fabry disease

Day 30 discussion

Action: For discussion

Endocrinology-Gynaecology-Fertility-Metabolism

3.3.7. Darvadstrocel - Orphan - EMEA-001561-PIP01-13-M01

Takeda Pharma A/S; Anal fistula

Day 30 discussion

Action: For discussion

Gastroenterology-Hepatology

3.3.8. Autologous CD34+ cells transduced with lentiviral vector encoding the human beta-globin gene - Orphan - EMEA-001933-PIP01-16-M01

Orchard Therapeutics (Europe) Ltd; Beta-thalassemia major and intermedia / Treatment of beta-thalassemia major and intermedia

Day 30 discussion

Action: For discussion

Haematology-Hemostaseology

3.3.9. Human fibrinogen concentrate - EMEA-001931-PIP01-16-M02

Biotest AG; Treatment of congenital fibrinogen deficiency

Day 30 discussion

Action: For discussion

Haematology-Hemostaseology

3.3.10. Roxadustat - EMEA-001557-PIP01-13-M04

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

Day 30 discussion

Action: For discussion

Haematology-Hemostaseology

3.3.11. Tofacitinib citrate - EMEA-000576-PIP01-09-M12

Pfizer Europe MA EEIG; Treatment of chronic idiopathic arthritis (including rheumatoid arthritis, psoriatic arthritis, ankylosing spondylitis and juvenile idiopathic arthritis) / Juvenile idiopathic arthritis

Day 30 discussion

Action: For discussion

Immunology-Rheumatology-Transplantation

3.3.12. Upadacitinib - EMEA-001741-PIP04-17-M01

AbbVie Ltd; Treatment of atopic dermatitis

Day 30 discussion

Action: For discussion

Immunology-Rheumatology-Transplantation / Dermatology

3.3.13. Tenofovir alafenamide / emtricitabine / cobicistat / elvitegravir - EMEA-001460-PIP01-13-M0

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

Day 30 discussion

Action: For discussion

Infectious Diseases

3.3.14. Eptinezumab - EMEA-002243-PIP01-17-M01

H. Lundbeck A/S; Prevention of migraine headaches / Prophylaxis of migraine

Day 30 discussion

Action: For discussion

Neurology

3.3.15. Erenumab - EMEA-001664-PIP02-15-M04

Novartis Europharm Limited; Prevention of migraine headaches / Prophylaxis of migraine

Day 30 discussion

Action: For discussion

Neurology

3.3.16. Eslicarbazepine acetate - EMEA-000696-PIP02-10-M07

BIAL - Portela & Ca, SA; Treatment of epilepsy with partial onset seizures / Treatment of epilepsy with partial onset seizures with eslicarbazepine acetate (ESL) as adjunctive therapy / Treatment of epilepsy with partial onset seizures with eslicarbazepine acetate (ESL) as monotherapy

Day 30 discussion

Action: For discussion

Neurology

3.3.17. Humanised anti-IL-6 receptor (IL-6R) monoclonal antibody (INN: satralizumab) - Orphan - EMEA-001625-PIP01-14-M05

Roche Registration GmbH; Treatment of neuromyelitis optica

Day 30 discussion

Action: For discussion

Neurology

3.3.18. Meningococcal group Y oligosaccharides conjugated to Corynebacterium diphtheriae CRM197 protein (MenABCWY) / recombinant *Neisseria meningitis* group B Protein 961c / recombinant *Neisseria meningitis* group B Protein 287- 953 / meningococcal group C oligosaccharides conjugated to *Corynebacterium diphtheriae* CRM197 protein / meningococcal group A oligosaccharides conjugated to *Corynebacterium diphtheriae* CRM197 protein / Outer Membrane Vesicles (OMV) from *Neisseria meningitidis* Strain NZ 98/254 / meningococcal group W-135 oligosaccharides conjugated to *Corynebacterium diphtheriae* CRM197 protein / recombinant *Neisseria meningitis* group B Protein 936-741 - EMEA-001260-PIP01-11-M01

GSK Vaccines s.r.l.; A39.9 Meningococcal infection in adults and paediatrics patients

Day 30 discussion

Action: For discussion

Vaccines

3.3.19. *Neisseria meningitidis* serogroup B recombinant lipoprotein (rLP2086; subfamily B; Escherichia coli) / *Neisseria meningitidis* serogroup B recombinant lipoprotein (rLP2086; subfamily A; Escherichia coli) - EMEA-001037-PIP02-11-M07

Pfizer Europe MA EEIG; Invasive meningococcal disease caused by *Neisseria meningitidis* serogroup B

Day 30 discussion

Action: For discussion

Vaccines

3.3.20. Monovalent, recombinant, replication-incompetent human adenovirus serotype 26-vectored vaccine encoding the pre-fusion conformation-stabilised F protein derived from the RSV A2 strain - EMEA-002172-PIP02-17-M01

Janssen-Cilag International NV; Prevention of lower respiratory tract disease caused by respiratory syncytial virus (RSV)

Day 30 discussion

Action: For discussion

Vaccines / Infectious Diseases

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 20X20for 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. Selective estrogen receptor degrader - EMEA-02-2020

AstraZeneca AB; The classes of oestrogen receptor modulator medicinal products for treatment of breast malignant neoplasms / Treatment of oestrogen receptor positive, locally advanced or metastatic breast cancer

Action: For adoption

6.1.2. Brolucizumab - EMEA-03-2020

Novartis Europharm Ltd; All classes of medicinal products for treatment of age-related macular degeneration and diabetic macular oedema/ Visual impairment due to diabetic macular edema

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

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

PDCO Chair: Koen Norga

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

9.3. 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)

Action: For information

9.5. Cooperation with International Regulators

9.5.1. Action: For information ICH S11 – Guideline on nonclinical safety testing in support of development of paediatric pharmaceuticals S11

PDCO Member: Karen van Malderen

Action: For adoption

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) – follow up from the Helsinki meeting 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

9.8.1. Marketing authorisation applications (MAA) forecast for 2020 – planning update dated Q1 2020

Action: For information

10. Any other business

No items

11. Breakout sessions

No items

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/