23 March 2021
EMA/PDCO/129227/2021
Human Medicines Division

Paediatric Committee (PDCO)

Agenda for the meeting on 23-26 March 2021

Chair: Koenraad Norga – Vice-Chair: Sabine Scherer

23 March 2021, 14:00- 19:00

24 March 2021, 08:30- 19:00

25 March 2021, 08:30- 19:00

26 March 2021, 08:30- 13:00

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 March 2021. See 23-26 March 2021 PDCO minutes (to be published post April 2021 PDCO meeting).

1.2. Adoption of agenda

PDCO agenda for 23-26 March 2021

1.3. Adoption of the minutes

PDCO minutes for 23-26 February 2021

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. RAAV8-hUGT1A1 - Orphan - EMEA-002021-PIP01-16

GENETHON; Treatment of Crigler-Najjar syndrome / Treatment of Severe Crigler-Najjar syndrome requiring phototherapy

Day 120 opinion

Action: For adoption

Endocrinology-Gynaecology-Fertility-Metabolism

2.1.2. Vedolizumab - EMEA-000645-PIP04-20

Active pouchitis (in patients who underwent proctocolectomy and ileal-pouch anal anastomosis for ulcerative colitis, familial adenomatous polyposis, and other underlying conditions for which construction of a pouch was medically indicated) / Active pouchitis (in patients who underwent proctocolectomy and ileal-pouch anal anastomosis for ulcerative colitis)

Day 120 opinion

Action: For adoption

Gastroenterology-Hepatology

2.1.3. Pegfilgrastim - EMEA-002671-PIP02-20

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

Day 120 opinion

Action: For adoption

Haematology-Hemostaseology

2.1.4. Human anti-interleukin-15 (IL-15) monoclonal antibody - EMEA-002775-PIP01-20

Treatment of coeliac disease

Day 120 opinion

Action: For adoption

Gastroenterology-Hepatology

2.1.5. Edaravone - Orphan - EMEA-002785-PIP01-20

Treeway B.V.; Amyotrophic lateral sclerosis

Day 120 opinion

Action: For adoption

Neurology

2.1.6. Respiratory syncytial virus stabilised prefusion F subunit vaccine (RSVpreF, PF-06928316) - EMEA-002795-PIP01-20

Prevention of lower respiratory tract disease caused by respiratory syncytial virus via maternal immunisation / Prevention of RSV-associated medically attended lower respiratory tract illness (MA-LRTI) and/or RSV associated severe MA-LRTI in neonates and infants by active immunisation of pregnant adolescents

Day 120 opinion

Action: For adoption

Vaccines

2.1.7. EMEA-002948-PIP01-20

Treatment of dilated cardiomyopathy

Day 60 opinion

Action: For adoption

Cardiovascular Diseases

2.1.8. Anti-CD40L humanized monoclonal antibody - EMEA-002945-PIP01-20

Treatment of Sjogren's Syndrome

Day 60 opinion

Action: For adoption

Immunology-Rheumatology-Transplantation

2.1.9. Reparixin - Orphan - EMEA-001693-PIP02-20

Dompé farmaceutici S.p.A.; Treatment of COVID-19 pneumonia / Treatment of severe COVID-19 pneumonia

Day 60 opinion

Action: For adoption

Infectious Diseases

2.1.10. EMEA-002943-PIP01-20

Mature B-Cell malignancies

Day 60 opinion

Action: For adoption

Oncology

2.1.11. Pembrolizumab / quavonlimab - EMEA-002949-PIP01-20

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

Day 60 opinion

Action: For adoption

Oncology

2.1.12. Recombinant protein derived from the SARS CoV2 prefusion Spike delta TM protein adjuvanted with AS03 - EMEA-002915-PIP01-20

Prevention of Covid-19

Day 90 opinion

Action: For adoption

Vaccines

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. Simoctocog alfa - EMEA-C-001024-PIP01-10-M02

Octapharma Pharmazeutika Produktionsges.m.b.H.; Treatment of haemophilia A (congenital Factor VIII deficiency)

Day 60 opinion

Action: For adoption

Haematology-Hemostaseology

2.2.2. Lenvatinib - EMEA-C3-001119-PIP02-12-M07

Eisai GmbH; Treatment of papillary thyroid cancer/ Treatment of follicular thyroid cancer/ Treatment of osteosarcoma

Day 60 letter

Action: For adoption

Oncology

2.3. Opinions on Modification of an Agreed Paediatric Investigation Plan

2.3.1. Pegcetacoplan - Orphan - EMEA-002600-PIP01-19-M01

Apellis Ireland Limited; Paroxysmal Nocturnal Haemoglobinuria

Day 60 opinion

Action: For adoption

2.3.2. Remimazolam - EMEA-001880-PIP02-19-M02

PAION Deutschland GmbH; Sedation, General anesthesia / Sedation of mechanically ventilated patients / Sedation for short procedures

Day 60 opinion

Action: For adoption

Anaesthesiology

2.3.3. Macitentan - Orphan - EMEA-001032-PIP01-10-M03

Janssen-Cilag International NV; Treatment of systemic sclerosis / Treatment of idiopathic pulmonary fibrosis / Treatment of pulmonary arterial hypertension

Day 60 opinion

Action: For adoption

Cardiovascular Diseases

2.3.4. Ticagrelor - EMEA-000480-PIP01-08-M14

AstraZeneca AB; Prevention of thromboembolic events

Day 60 opinion

Action: For adoption

Cardiovascular Diseases / Haematology-Hemostaseology

2.3.5. Spesolimab - EMEA-002475-PIP02-19-M01

Boehringer Ingelheim International GmbH; Prevention of Generalized Pustular Psoriasis / Treatment of Generalized Pustular Psoriasis / Treatment of patients with acute or chronic Generalized Pustular Psoriasis (GPP) and for the prevention of flares

Day 60 opinion

Action: For adoption

Dermatology

2.3.6. Cipaglucosidase alfa - Orphan - EMEA-002447-PIP01-18-M01

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

Day 60 opinion

Action: For adoption

Endocrinology-Gynaecology-Fertility-Metabolism

2.3.7. Pegunigalsidase alfa - Orphan - EMEA-001828-PIP01-15-M02

Chiesi Farmaceutici S.p.A.; Treatment of Fabry disease

Day 60 opinion

Action: For adoption

Endocrinology-Gynaecology-Fertility-Metabolism

2.3.8. Avacopan - Orphan - EMEA-002023-PIP01-16-M05

ChemoCentryx Ireland Ltd.; Treatment of anti-neutrophil cytoplasmic auto-antibody (ANCA)-associated vasculitis

Day 60 opinion

Action: For adoption

Immunology-Rheumatology-Transplantation

2.3.9. Doravirine - EMEA-001676-PIP01-14-M04

Merck Sharp & Dohme (Europe), Inc.; Treatment of human immunodeficiency virus-1 (HIV-1) infection / Antiretroviral therapy, in combination with other antiretroviral agents, for the treatment of HIV-1 infection in children aged from birth to 18 years

Day 60 opinion

Action: For adoption

Infectious Diseases

2.3.10. Rilpivirine / Dolutegravir - EMEA-001750-PIP01-15-M04

ViiV Healthcare UK Limited; Treatment of Human Immunodeficiency Virus type 1 (HIV-1) infection

Day 60 opinion

Action: For adoption

Infectious Diseases

2.3.11. Tenofovir alafenamide / emtricitabine / cobicistat / darunavir - EMEA-001825-PIP01-15-M03

Janssen-Cilag International NV; Treatment of human immunodeficiency virus type-1 (HIV-1) infection / Treatment of HIV-1 infection in paediatric subjects weighing 25 kg or more from 6 years of age

Day 60 opinion

Action: For adoption

Infectious Diseases

2.3.12. Tenofovir disoproxil fumarate / lamivudine / doravirine - EMEA-001695-PIP01-14-M04

Merck Sharp & Dohme (Europe), Inc.; Treatment of human immunodeficiency virus-1 (HIV-1) infection / Antiretroviral combination therapy, for the treatment of HIV-1 infection in adults and children aged 6 to 18 years

Day 60 opinion

Action: For adoption

Infectious Diseases

2.3.13. Ataluren - Orphan - EMEA-000115-PIP01-07-M11

PTC Therapeutics International, Limited; Treatment of dystrophinopathy / Treatment of nonsense-mutation dystrophinopathy

Day 60 opinion

Action: For adoption

Neurology

2.3.14. Brivaracetam - Orphan - EMEA-000332-PIP01-08-M16

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

Day 60 opinion

Action: For adoption

Neurology

2.3.15. Brivaracetam - Orphan - EMEA-000332-PIP02-17-M02

UCB Pharma S.A.; Treatment of Neonatal Seizures, Treatment of paediatric epilepsy syndromes / Treatment of Neonatal Seizures with adjunctive administration of brivaracetam, Monotherapy in patients 4 to 25 years of age with Childhood Absence Epilepsy (CAE) and Juvenile Absence Epilepsy (JAE)

Day 60 opinion

Action: For adoption

Neurology

2.3.16. Bumetanide - EMEA-001303-PIP01-12-M03

Les Laboratoires Servier; Autism Spectrum Disorder / Treatment of Autism Spectrum Disorder

Day 60 opinion

Action: For adoption

Neurology

2.3.17. Ganaxolone - Orphan - EMEA-002341-PIP01-18-M01

Marinus Pharmaceuticals Inc.; Cyclin-dependent kinase-like 5 (CDKL5) deficiency disorder / Adjunctive treatment of seizures in paediatric patients aged 6 months to < 18 years old with cyclin-dependent kinase-like 5 (CDKL5) deficiency disorder

Day 60 opinion

Action: For adoption

Neurology

2.3.18. Dostarlimab - EMEA-002463-PIP01-18-M01

GlaxoSmithKline (Ireland) Limited; Treatment of all conditions included in the category of malignant neoplasms (except haematopoietic and lymphoid malignancies) / Treatment of paediatric patients from birth to less than 18 years old with neuroblastoma and/or osteosarcoma

Day 60 opinion

Action: For adoption

Oncology

2.3.19. Isatuximab - EMEA-002205-PIP01-17-M02

Sanofi-Aventis Recherche & Développement; Treatment of malignant neoplasms of the haematopoietic and lymphoid tissue / Treatment of relapsed, refractory and newly-diagnosed acute myeloid leukemia in combination with standard treatment (chemotherapy) in paediatric patients from 28 days to less than 18 years of age / Treatment of relapsed, refractory and newly-diagnosed acute lymphoblastic leukemia in combination with standard treatment (chemotherapy) in paediatric patients from 28 days to less than 18 years of age

Day 60 opinion

Action: For adoption

Oncology

2.3.20. Lenvatinib - EMEA-001119-PIP03-19-M01

Eisai GmbH; Treatment of all conditions included in the category of malignant neoplasms except haematopoietic and lymphoid tissue neoplasms, papillary thyroid cancer, follicular thyroid cancer and osteosarcoma / Treatment of paediatric patients from 2 years to less than 18 years old with a relapsed or refractory solid malignant tumour including Ewing sarcoma/peripheral primitive neuroectodermal tumour, rhabdomyosarcoma and high-grade glioma

Day 60 opinion

Action: For adoption

Oncology

2.3.21. Niraparib (as tosylate monohydrate) - Orphan - EMEA-002268-PIP02-18-M01

GlaxoSmithKline (Ireland) Limited; Treatment of all conditions included in the category of malignant neoplasms (except haematopoietic and lymphoid malignancies) / Treatment of paediatric patients from birth to less than 18 years old with neuroblastoma and/or osteosarcoma

Day 60 opinion

Action: For adoption

Oncology

2.3.22. Obinutuzumab - Orphan - EMEA-001207-PIP01-11-M01

Roche Registration GmbH; Treatment of mature B-cell lymphoma / Treatment of acute lymphoblastic leukaemia

Day 60 opinion

Action: For adoption

Oncology

2.3.23. Ruxolitinib phosphate - EMEA-000901-PIP03-16-M02

Novartis Europharm Limited; Acute Graft versus Host Disease / Treatment of acute Graft versus Host Disease (aGvHD) in paediatric patients aged 28 days and above

Day 60 opinion

Action: For adoption

Oncology

2.3.24. Concentrate of proteolytic enzyme enriched in bromelain - Orphan - EMEA-000142-PIP02-09-M11

MediWound Germany GmbH; Treatment of burns of external body surfaces / Removal of eschar in deep partial and/or full thickness burns

Day 60 opinion

Action: For adoption

Other

2.3.25. Meloxicam / Bupivacaine - EMEA-002246-PIP01-17-M02

Heron Therapeutics B.V.; Acute postoperative pain

Day 60 opinion

Action: For adoption

Pain

2.3.26. Birch pollen extract (Betula verrucosa) - EMEA-001879-PIP01-15-M03

ALK-Abelló A/S; Treatment of allergic rhinitis / rhino-conjunctivitis / Treatment of tree pollen allergic rhinitis / rhino-conjunctivitis

Day 60 opinion

Action: For adoption

Pneumology - Allergology

2.3.27. Brexpiprazole - EMEA-001185-PIP01-11-M07

Otsuka Pharmaceutical Development & Commercialisation Europe GmbH; Treatment of schizophrenia from 13 to less than 18 years of age

Day 60 opinion

Action: For adoption

Psychiatry

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

2.6.1. Oxygen / Argon - EMEA-002921-PIP01-20

Treatment of acute ischaemic stroke

Correction of Opinion

Action: For adoption

Neurology

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. Brentuximab vedotin - EMEA-C5-000980-PIP01-10-M07

Takeda Pharma A/S; Treatment of Hodgkin lymphoma

Day 1 letter

Action: For information

Oncology

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. EMEA-002735-PIP01-19

Cardioplegia / For induction of cardioplegia in paediatric patients of all ages undergoing cardiac surgery to correct congenital heart malformation in operations requiring CPB support

Day 90 discussion

Action: For discussion

Cardiovascular Diseases

3.1.2. Sotatercept - EMEA-002756-PIP01-19

Pulmonary arterial hypertension

Day 90 discussion

Action: For discussion

Cardiovascular Diseases

3.1.3. Human plasma derived c1-inhibitor - EMEA-002818-PIP01-20

Treatment of hereditary angioedema (HAE)

Day 90 discussion

Action: For discussion

Immunology-Rheumatology-Transplantation

3.1.4. Iscalimab - EMEA-002842-PIP01-20

Prophylaxis of solid organ transplant rejection

Day 90 discussion

Action: For discussion

Immunology-Rheumatology-Transplantation

3.1.5. Ravulizumab - EMEA-001943-PIP03-20

Acetylcholine receptor-antibody positive generalized myasthenia gravis / Treatment of acetylcholine receptor-antibody positive generalized myasthenia gravis

Day 90 discussion

Action: For discussion

Neurology

3.1.6. Erdafitinib - EMEA-002042-PIP02-20

Treatment of all conditions included in the category of malignant neoplasms (except urothelial carcinoma, haematopoietic and lymphoid tissue neoplasms) / Treatment of locally advanced or metastatic solid tumors, including primary CNS tumors, harboring susceptible FGFR alterations in patients who have progressed following prior therapies and those who have no acceptable standard therapies

Day 90 discussion

Action: For discussion

Oncology

3.1.7. Talazoparib - EMEA-002066-PIP01-20

Treatment of Ewing sarcoma / Treatment of refractory or recurrent Ewing Sarcoma

Day 90 discussion

Action: For discussion

Oncology

3.1.8. Atropine - EMEA-002744-PIP01-19

Myopia / Treatment to slow myopia progression

Day 90 discussion

Action: For discussion

Ophthalmology

3.1.9. EMEA-002735-PIP03-20

Heart transplantation / Preservation of hearts prior to heart transplantation

Day 60 discussion

Action: For discussion

Cardiovascular Diseases

3.1.10. EMEA-002944-PIP01-20

Treatment of Type 2 Diabetes Mellitus

Day 60 discussion

Action: For discussion

Endocrinology-Gynaecology-Fertility-Metabolism

3.1.11. Peptide derivative of glucagon-like-peptide 1 and glucagon with fatty acid side chain - EMEA-002942-PIP01-20

Treatment of NASH/NAFLD with fibrosis

Day 60 discussion

Action: For discussion

Gastroenterology-Hepatology

3.1.12. Peptide derivative of glucagon-like-peptide 1 and glucagon with fatty acid side chain - EMEA-002942-PIP02-20

Treatment of obesity

Day 60 discussion

Action: For discussion

Gastroenterology-Hepatology

3.1.13. Marzeptacog alfa (activated) - Orphan - EMEA-002270-PIP03-20

Catalyst Biosciences, Inc.; Treatment of haemophilia A / Treatment of bleeding episodes in patients with congenital haemophilia A with inhibitors to Factor VIII

Day 60 discussion

Action: For discussion

Haematology-Hemostaseology

3.1.14. Marzeptacog alfa (activated) - Orphan - EMEA-002270-PIP04-20

Catalyst Biosciences, Inc.; Treatment of haemophilia B / Treatment of bleeding episodes in patients with congenital HB with inhibitors to Factor IX

Day 60 discussion

Action: For discussion

Haematology-Hemostaseology

3.1.15. Baricitinib - EMEA-001220-PIP08-20

Treatment of alopecia areata

Day 60 discussion

Action: For discussion

Immunology-Rheumatology-Transplantation

3.1.16. Exebacase - EMEA-002947-PIP01-20

Treatment of *Staphylococcus aureus* bloodstream infections (bacteraemia)

Day 60 discussion

Action: For discussion

Infectious Diseases

3.1.17. Regdanvimab - EMEA-002961-PIP01-21

Treatment of coronavirus disease 2019 (COVID-19)

Day 60 discussion

Action: For discussion

Infectious Diseases

3.1.18. EMEA-002984-PIP01-21

Treatment of onychomycosis / Treatment of distal subungual onychomycosis

Day 60 discussion

Action: For discussion

Infectious Diseases

3.1.19. Lutetium (177Lu) oxodotreotide - Orphan - EMEA-002950-PIP01-20

Advanced Accelerator Applications; Gastroenteropancreatic neuroendocrine tumours (GEP-NETs) / Treatment of unresectable or metastatic, progressive, well differentiated (G1 and G2), somatostatin receptor positive gastroenteropancreatic neuroendocrine tumours (GEP-NETs) in adolescent patients (12 years and older)

Day 60 discussion

Action: For discussion

Oncology

3.1.20. Apitegromab - Orphan - EMEA-002951-PIP01-20

Scholar Rock, Inc.; Spinal muscular atrophy

Day 60 discussion

Action: For discussion

Other / Neurology

3.1.21. EMEA-002946-PIP01-20

Treatment of Major Depressive Disorder (MDD) / Rapid reduction of depressive symptoms, in conjunction with comprehensive standard of care (SoC), in adolescents with MDD who have suicidal ideation with intent

Day 60 discussion

Action: For discussion

Psychiatry

3.1.22. EMEA-002958-PIP01-21

Treatment of hypertrophic cardiomyopathy

Day 30 discussion

Action: For discussion

Cardiovascular Diseases

3.1.23. EMEA-002962-PIP01-21

Treatment of elevated cholesterol / Primary hypercholesterolemia (non-familial) and mixed dyslipidemia / Established ASCVD / Heterozygous familial hypercholesterolemia (HeFH) / Homozygous familial hypercholesterolemia (HoFH)

Day 30 discussion

Action: For discussion

Endocrinology-Gynaecology-Fertility-Metabolism

3.1.24. Drospirenone - EMEA-001495-PIP02-21

Treatment of endometriosis

Day 30 discussion

Action: For discussion

Endocrinology-Gynaecology-Fertility-Metabolism

3.1.25. Poly(oxy-1,2-ethanediyl), alpha-hydro-omega-methoxy, ether with N-[[[2-[[6-[[1-[3-[[3-(2,3-dihydroxypropoxy)propyl]amino]-3-oxopropyl]-2,5-dioxo-3-pyrrolidinyl]thio]hexyl]amino]ethyl]amino]carbonyl]-2-methylalanyl-teriparatide (2:1) - Orphan - EMEA-002955-PIP01-21

Ascendis Pharma Bone Diseases A/S; Hypoparathyroidism / Treatment of hypoparathyroidism in patients from birth to less than 18 years of age

Day 30 discussion

Action: For discussion

Endocrinology-Gynaecology-Fertility-Metabolism

3.1.26. Tildacerfont - Orphan - EMEA-002970-PIP01-21

Spruce Biosciences, Inc.; Treatment of congenital adrenal hyperplasia

Day 30 discussion

Action: For discussion

Endocrinology-Gynaecology-Fertility-Metabolism

3.1.27. Nangibotide - EMEA-002953-PIP01-21

Septic shock / Treatment of septic shock in children

Day 30 discussion

Action: For discussion

Infectious Diseases

3.1.28. EMEA-002964-PIP01-21

Treatment and decreased transmission of coronavirus disease 2019 (COVID-19) / Prophylaxis of SARS-CoV-2 infection

Day 30 discussion

Action: For discussion

Infectious Diseases

3.1.29. EMEA-002965-PIP01-21

Treatment and decreased transmission of coronavirus disease 2019 (COVID-19) / Prophylaxis of SARS-CoV-2 infection

Day 30 discussion

Action: For discussion

Infectious Diseases

3.1.30. Tosatoxumab - Orphan - EMEA-002506-PIP03-21

Aridis Pharmaceuticals Inc; *Staphylococcus aureus* pneumonia

Day 30 discussion

Action: For discussion

Infectious Diseases / Pneumology - Allergology

3.1.31. Naproxen sodium / Sumatriptan - EMEA-002959-PIP01-21

Acute treatment of migraine attacks

Day 30 discussion

Action: For discussion

Neurology

3.1.32. Savolitinib - EMEA-002627-PIP02-21

Treatment of malignant renal neoplasms

Day 30 discussion

Action: For discussion

Oncology

3.1.33. Synthetic hypericin - Orphan - EMEA-002956-PIP01-21

Soligenix NL B.V; Cutaneous T-Cell Lymphoma

Day 30 discussion

Action: For discussion

Oncology

3.1.34. EMEA-002895-PIP02-21

Treatment of macular oedema due to central or tributary (branch) retinal vein occlusion / Treatment of diabetic retinopathy / Treatment of choroidal neovascularisation

Day 30 discussion

Action: For discussion

Ophthalmology

3.1.35. Zorecimeran - EMEA-002986-PIP01-21

Prevention of coronavirus disease (COVID-19)

Day 30 discussion

Action: For discussion

Vaccines / Infectious Diseases

3.1.36. Human SARS-CoV-2 immunoglobulin - EMEA-002911-PIP01-20

Treatment of coronavirus disease 2019 (COVID-19) / Treatment of hospitalised patients with COVID-19 disease

Day 30 discussion

Action: For discussion

Other

3.1.37. Human SARS-CoV-2 immunoglobulin - EMEA-002912-PIP01-20

Treatment of coronavirus disease 2019 (COVID-19)

Day 30 discussion

Action: For discussion

Other

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. Teduglutide - EMEA-C-000482-PIP01-08-M06

Takeda Pharmaceuticals International AG; Treatment of short bowel syndrome

Day 30 discussion

Action: For discussion

Gastroenterology-Hepatology

3.2.2. Baloxavir marboxil - EMEA-C2-002440-PIP01-18-M01

Roche Registration GmbH; Treatment of influenza

Day 30 discussion

Action: For discussion

Infectious Diseases

3.2.3. Afatinib - EMEA-C-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)

Day 30 discussion

Action: For discussion

Oncology

3.3. Discussions on Modification of an Agreed Paediatric Investigation Plan

3.3.1. Liquid ethalonic 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-M05

LEGACY HEALHCARE; Treatment of alopecia

Day 30 discussion

Action: For discussion

Dermatology

3.3.2. Denosumab - EMEA-000145-PIP02-12-M04

Amgen Europe B.V.; Treatment of Osteoporosis / Treatment of osteogenesis imperfecta / Treatment of glucocorticoid induced osteoporosis

Day 30 discussion

Action: For discussion

Endocrinology-Gynaecology-Fertility-Metabolism

3.3.3. Sotagliflozin - EMEA-001517-PIP02-14-M03

Guidehouse Germany GmbH; Treatment of type 1 diabetes mellitus / Treatment of type 1 diabetes mellitus

Day 30 discussion

Action: For discussion

Endocrinology-Gynaecology-Fertility-Metabolism

3.3.4. Linaclotide - EMEA-000927-PIP01-10-M06

Allergan Pharmaceuticals International Limited; Functional constipation in children

Day 30 discussion

Action: For discussion

Gastroenterology-Hepatology

3.3.5. Betibeglogene autotemcel - Orphan - EMEA-001665-PIP01-14-M05

bluebird bio (Netherlands) B.V.; Treatment of β-thalassaemia / Treatment of beta-thalassaemia major and severe intermedia

Day 30 discussion

Action: For discussion

Haematology-Hemostaseology

3.3.6. Setmelanotide - Orphan - EMEA-002209-PIP01-17-M02

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

3.3.7. Venetoclax - Orphan - EMEA-002018-PIP02-16-M04

AbbVie Ltd; Treatment of haematopoietic and lymphoid malignant neoplasms / Treatment of solid tumour malignant neoplasms / Treatment of a specific paediatric hematopoietic and lymphoid malignant neoplasm or of a solid malignant neoplasm as agreed by PDCO, in patients from 1 month to 18 years of age

Day 30 discussion

Action: For discussion

Oncology / Haematology-Hemostaseology

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 submission of applications with start of procedure 23 March 2021 for Nomination of Rapporteur and Peer reviewer

Action: For adoption

4.2. Nomination of Rapporteur for requests of confirmation on the applicability of the EMA decision on class waiver

Action: For adoption

4.3. Nominations for other activities

Action: For adoption

5. Scientific Advice Working Party (SAWP) and Paediatric Committee (PDCO) Interaction

Disclosure of information related to this section cannot be released at the present time as it is deemed

to contain commercially confidential information.

6. Discussion on the applicability of class waivers

Disclosure of some information related to this section cannot be released at the present time as it is deemed to contain commercially confidential information.

6.1. Discussions on the applicability of class waiver for products

6.1.1. Semaglutide - EMEA-01-2021

Novo Nordisk; All classes of medicinal products for treatment of Alzheimer's disease / Treatment of mild cognitive impairment and mild dementia, both of the Alzheimer's type

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. Roll-out of WebEx to PDCO / EMA

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)

No items

9.4.2. Feedback from EMA/EUnetHTA meeting on extrapolation

Action: For discussion

9.5. Cooperation with International Regulators

9.5.1. Paediatric oncology common commentary

Action: For adoption

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

No items

9.7. PDCO work plan

No items

9.8. Planning and reporting

No items

10. Any other business

10.1. COVID -19 update

Action: For information

10.2. Re-engineered ITF

Action: For information

10.3. Update on CONSIGN project - EMA draft pregnancy strategy

Action: For information

10.4. EMA Business Pipeline activity and Horizon scanning

Q1/2021 Update of the Business Pipeline report for the human scientific committees

Action: For information

10.5. Update on the revision of the Regulation

Action: For information

11. Breakout sessions

11.1. Paediatric oncology

Action: For discussion on Tuesday, 12:00 - 13:00

11.2. Neonatology

Action: For discussion on Thursday, 13:00 - 14:00

11.3. Revision of the Regulation

Action: For discussion on Tuesday, 11:00-12:00

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/