16 July 2020
EMA/PDCO/369320/2020
Human Medicines Division

Paediatric Committee (PDCO)

Agenda for the meeting on 21-24 July 2020

Chair: Koenraad Norga – Vice-Chair: Sabine Scherer

21 July 2020, 14:00- 19:00, Virtual meeting

22 July 2020, 08:30- 19:00, Virtual meeting

23 July 2020, 08:30- 19:00, Virtual meeting

24 July 2020, 08:30- 13:00, 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).

Official address Domenico Scarlattilaan 6 ● 1083 HS Amsterdam ● The Netherlands
Address for visits and deliveries Refer to www.ema.europa.eu/how-to-find-us
Send us a question Go to www.ema.europa.eu/contact **Telephone** +31 (0)88 781 6000 An agency of the European Union

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 21-24 July 2020. See July 2020 PDCO minutes (to be published post July 2020 PDCO meeting).

1.2. Adoption of agenda

PDCO agenda for 21-24 July 2020

1.3. Adoption of the minutes

PDCO minutes for extraordinary PDCO virtual meeting 14 May 2020

PDCO minutes for 23-26 June 2020

PDCO minutes for extraordinary PDCO virtual meeting 9 July 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. Bilayer, engineered, collagen hydrogel-based skin graft composed of autologous keratinocytes and fibroblasts - Orphan - EMEA-002699-PIP01-19

CUTISS AG; Treatment of burns

Day 120 opinion

Action: For adoption

Dermatology

2.1.2. 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 120 opinion

Action: For adoption

Endocrinology-Gynaecology-Fertility-Metabolism

2.1.3. Rebisufligene etisparvovec - Orphan - EMEA-002206-PIP02-19

Abeona Therapeutics Inc.; Treatment of mucopolysaccharidosis type IIIA (Sanfilippo A syndrome) in children from birth to less than 18 years of age

Day 120 opinion

Action: For adoption

Endocrinology-Gynaecology-Fertility-Metabolism

2.1.4. Odevixibat - Orphan - EMEA-002054-PIP02-18

Albireo AB; Biliary atresia

Day 120 opinion

Action: For adoption

Gastroenterology-Hepatology

2.1.5. Relamorelin - EMEA-002323-PIP02-19

Gastroparesis

Day 120 opinion

Action: For adoption

Gastroenterology-Hepatology

2.1.6. 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 intermedia and major / Treatment of transfusion-dependent beta-thalassemia

Day 120 opinion

Action: For adoption

Haematology-Hemostaseology

2.1.7. Mitapivat - EMEA-002684-PIP01-19

Pyruvate kinase deficiency / Treatment of paediatric patients with Pyruvate kinase deficiency

Day 120 opinion

Action: For adoption

Haematology-Hemostaseology

2.1.8. 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 120 opinion

Action: For adoption

Haematology-Hemostaseology

2.1.9. Artesunate - Orphan - EMEA-002710-PIP01-19

Amivas Ireland Ltd; Malaria

Day 120 opinion

Action: For adoption

Infectious Diseases

2.1.10. Diroximel - EMEA-002685-PIP02-19

Treatment of multiple sclerosis

Day 120 opinion

Action: For adoption

Neurology

2.1.11. EMEA-002631-PIP01-19

Treatment of acute myeloid leukaemia

Day 120 opinion

Action: For adoption

Oncology

2.1.12. Nivolumab / relatlimab - EMEA-002727-PIP01-19

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

Day 120 opinion

Action: For adoption

Oncology

2.1.13. Emixustat - EMEA-002581-PIP01-19

Stargardt disease

Day 120 opinion

Action: For adoption

Ophthalmology

2.1.14. Alpelisib - EMEA-002016-PIP03-19

PIK3CA related overgrowth spectrum (PROS)

Day 120 opinion

Action: For adoption

Other

2.1.15. Anti-neonatal Fc receptor human monoclonal antibody - Orphan - EMEA-002559-PIP03-19

Momenta Pharmaceuticals, Inc.; Autoimmune haemolytic aneamia

Day 120 opinion

Action: For adoption

Other

2.1.16. Acetylsalicylic acid / rosuvastatin - EMEA-002831-PIP01-20

Prevention of cardiovascular events

Day 60 opinion

Action: For adoption

Cardiovascular Diseases

2.1.17. Bisoprolol / ramipril - EMEA-002794-PIP01-20

Treatment of heart failure / Treatment of coronary artery disease / Treatment of hypertension

Day 60 opinion

Action: For adoption

Cardiovascular Diseases

2.1.18. Linerixibat - EMEA-002800-PIP01-20

Treatment of primary biliary cholangitis

Day 60 opinion

Action: For adoption

Gastroenterology-Hepatology

2.1.19. EMEA-002825-PIP01-20

Sicca syndrome (Sjögren's)

Day 60 opinion

Action: For adoption

Immunology-Rheumatology-Transplantation

2.1.20. Fruquintinib - EMEA-002784-PIP01-20

Treatment of colorectal carcinoma

Day 60 opinion

Action: For adoption

Oncology

2.1.21. Prolgolimab - EMEA-002792-PIP01-20

Non-small cell neoplasms malignant of the respiratory tract cell-type specified / Treatment of metastatic non-squamous non-small cell lung cancer

Day 60 opinion

Action: For adoption

Oncology

2.1.22. EMEA-002798-PIP01-20

Treatment of squamous carcinoma of the anal canal (SCAC)

Day 60 opinion

Action: For adoption

Oncology

2.1.23. Faricimab - EMEA-002817-PIP03-20

Diabetic retinopathy

Day 60 opinion

Action: For adoption

Ophthalmology

2.1.24. Faricimab - EMEA-002817-PIP04-20

Retinal vein occlusion

Day 60 opinion

Action: For adoption

Ophthalmology

2.1.25. Ranibizumab - EMEA-002832-PIP01-20

Diabetic retinopathy

Day 60 opinion

Action: For adoption

Ophthalmology

2.1.26. Dronabinol - EMEA-000643-PIP02-20

Treatment of spasticity

Day 60 opinion

Action: For adoption

Other / Neurology

2.1.27. Tezepelumab - EMEA-001613-PIP02-20

Chronic rhinosinusitis with nasal polyps

Day 60 opinion

Action: For adoption

Oto-rhino-laryngology

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. Idarucizumab - EMEA-C-001438-PIP01-13-M01

Boehringer Ingelheim International GmbH; Prevention of dabigatran associated haemorrhage

Day 60 opinion

Action: For adoption

Cardiovascular Diseases / Haematology-Hemostaseology

2.2.2. Eftrenonacog alfa - EMEA-C-000914-PIP01-10-M05

Swedish Orphan Biovitrum AB (publ); Treatment of hereditary factor IX deficiency

Day 60 opinion

Action: For adoption

Haematology-Hemostaseology

2.2.3. Avapritinib - EMEA-C1-002358-PIP02-18-M01

Blueprint Medicines (Netherlands) B.V.; Treatment of all conditions included in the category of malignant neoplasms (except haematopoietic and lymphoid tissue neoplasms)

Day 60 letter

Action: For adoption

Oncology

2.2.4. Glucarpidase - EMEA-C-001391-PIP01-12

Protherics Medicines Development BV; Treatment of methotrexate toxicity

Day 60 opinion

Action: For adoption

Oncology

2.2.5. Pazopanib - EMEA-C-000601-PIP01-09-M06

Novartis Europharm Limited; Treatment of Ewing sarcoma family of tumours

Day 60 opinion

Action: For adoption

Oncology

2.2.6. Clostridium Botulinum neurotoxin type A (150 kD), free of complexing proteins - EMEA-C-001039-PIP02-12-M04

Merz Pharmaceuticals GmbH; Treatment of sialorrhea

Day 30 opinion

Action: For discussion

Neurology

2.2.7. Pneumococcal polysaccharide serotype 1 – diphtheria CRM197 conjugate / pneumococcal polysaccharide serotype 3 – diphtheria CRM197 conjugate / pneumococcal polysaccharide serotype 4 – diphtheria CRM197 conjugate / pneumococcal polysaccharide serotype 5 – diphtheria CRM197 conjugate /

pneumococcal polysaccharide serotype 6A – diphtheria CRM197 conjugate / pneumococcal polysaccharide serotype 6B – diphtheria CRM197 conjugate / pneumococcal polysaccharide serotype 7F – diphtheria CRM197 conjugate / pneumococcal polysaccharide serotype 9V – diphtheria CRM197 conjugate / pneumococcal polysaccharide serotype 14 – diphtheria CRM197 conjugate / pneumococcal polysaccharide serotype 18C – diphtheria CRM197 conjugate / pneumococcal polysaccharide serotype 19A – diphtheria CRM197 conjugate / pneumococcal polysaccharide serotype 19F – diphtheria CRM197 conjugate / pneumococcal polysaccharide serotype 22F – diphtheria CRM197 conjugate / pneumococcal polysaccharide serotype 23F – diphtheria CRM197 conjugate / pneumococcal polysaccharide serotype 33F – diphtheria CRM197 conjugate (15-valent pneumococcal polysaccharide conjugate vaccine [V114]) - EMEA-C1-002215-PIP01-17-M02

Merck Sharp & Dohme (Europe), Inc.; Prevention of disease caused by *Streptococcus pneumoniae*

Day 60 letter

Action: For adoption

Vaccines

2.2.8. Spheroids of human autologous matrix-associated chondrocytes - EMEA-C-001264-PIP01-12-M02

CO.DON AG; Treatment of symptomatic articular cartilage defects of the femoral condyle and the patella of the knee (International cartilage repair society (ICRS) grade III or IV) with defect sizes up to 10 cm^2

Day 30 opinion

Action: For discussion

Other

2.2.9. Delamanid - EMEA-C-001113-PIP01-10-M06

Otsuka Pharmaceutical Development & Commercialisation Europe GmbH; Treatment of multi drug resistant tuberculosis

Day 30 opinion

Action: For discussion

Infectious Diseases

2.3. Opinions on Modification of an Agreed Paediatric Investigation Plan

2.3.1. Tadalafil - EMEA-000452-PIP02-10-M06

Eli Lilly and Company Ltd; Benign prostatic hyperplasia / Pulmonary arterial hypertension / Treatment of persistent pulmonary hypertension of the newborn / Treatment of pulmonary arterial hypertension

Day 60 opinion

Action: For adoption

Cardiovascular Diseases

2.3.2. Exenatide - EMEA-000689-PIP01-09-M10

AstraZeneca AB; Non-insulin dependent diabetes mellitus (excluding treatment with thiazolidinediones) / Non-insulin dependent diabetes mellitus (treatment including thiazolidinediones) / Non-insulin dependent diabetes mellitus - in combination with insulin (with or without oral antidiabetics) / Treatment of Type 2 diabetes mellitus

Day 60 opinion

Action: For adoption

Endocrinology-Gynaecology-Fertility-Metabolism

2.3.3. Cenicriviroc - EMEA-001999-PIP02-17-M01

Allergan Pharmaceuticals International Limited; NASH with Stage 2-3 fibrosis

Day 60 opinion

Action: For adoption

Gastroenterology-Hepatology

2.3.4. Odevixibat - Orphan - EMEA-002054-PIP01-16-M02

Albireo AB; Progressive familial intrahepatic cholestasis (PFIC)

Day 60 opinion

Action: For adoption

Gastroenterology-Hepatology

2.3.5. Potassium chloride / sodium chloride / citric acid, anhydrous / sodium citrate / simeticone / sodium sulphate, anhydrous / macrogol 4000 - EMEA-001356-PIP02-12-M03

Alfasigma S.p.A.; Any clinical procedures requiring a clean bowel e.g. bowel endoscopy or radiology

Day 60 opinion

Action: For adoption

Gastroenterology-Hepatology

2.3.6. Tofacitinib - EMEA-000576-PIP03-12-M04

Pfizer Europe MA EEIG; Treatment of ulcerative colitis

Day 60 opinion

Action: For adoption

Gastroenterology-Hepatology

2.3.7. Vedolizumab - EMEA-000645-PIP01-09-M07

Takeda Pharma A/S; Crohn's Disease / Ulcerative colitis / Adults and paediatrics

Day 60 opinion

Action: For adoption

Gastroenterology-Hepatology

2.3.8. Upadacitinib - EMEA-001741-PIP01-14-M03

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

Day 60 opinion

Action: For adoption

Immunology-Rheumatology-Transplantation

2.3.9. Treosulfan - Orphan - EMEA-000883-PIP01-10-M05

medac Gesellschaft für klinische Spezialpräparate mbH; Conditioning treatment prior to haematopoietic progenitor cell transplantation

Day 60 opinion

Action: For adoption

Immunology-Rheumatology-Transplantation / Oncology

2.3.10. Avibactam / ceftazidime - EMEA-001313-PIP01-12-M09

Pfizer Europe MA EEIG; Treatment of bacterial infections / For the treatment of complicated urinary tract infections / For the treatment of complicated intra-abdominal infections / For the treatment of pneumonia / For the treatment of infections due to aerobic Gram-negative organisms

Day 60 opinion

Action: For adoption

Infectious Diseases

2.3.11. Bedaquiline (fumarate) - Orphan - EMEA-000912-PIP01-10-M05

Janssen-Cilag International NV; Treatment of multi-drug resistant tuberculosis

Day 60 opinion

Action: For adoption

Infectious Diseases

2.3.12. Cabotegravir - EMEA-001418-PIP01-13-M02

ViiV Healthcare UK Limited; Treatment of human immunodeficiency virus (HIV-1) infection / Treatment of human immunodeficiency virus (HIV-1) infection, in combination with other antirectoviral agents

Day 60 opinion

Action: For adoption

Infectious Diseases

2.3.13. Cefiderocol - EMEA-002133-PIP01-17-M01

Shionogi B.V.; Treatment of infections due to aerobic Gram-negative bacteria

Day 60 opinion

Action: For adoption

Infectious Diseases

2.3.14. Lamivudine / dolutegravir - EMEA-001940-PIP01-16-M03

ViiV Healthcare UK Limited; Treatment of human immunodeficiency virus (HIV - 1) infection / Treatment of human immunodeficiency virus (HIV - 1) infection

Day 60 opinion

Action: For adoption

Infectious Diseases

2.3.15. Maribavir - Orphan - EMEA-000353-PIP02-16-M01

Shire Pharmaceuticals Ireland Limited; Treatment of cytomegalovirus (CMV) infection / Treatment of CMV infection in paediatric patients who have undergone a haematopoietic stem cell transplant (HSCT) or solid organ transplant (SOT)

Day 60 opinion

Action: For adoption

Infectious Diseases

2.3.16. Oseltamivir phosphate - EMEA-000365-PIP01-08-M11

Roche Registration GmbH; Treatment and prevention of influenza / Treatment and prevention of influenza in healthy and immunocompromised patients from birth to less than

18 years of age

Day 60 opinion

Action: For adoption

Infectious Diseases

2.3.17. Pretomanid - Orphan - EMEA-002115-PIP01-17-M02

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

Day 60 opinion

Action: For adoption

Infectious Diseases

2.3.18. Rilpivirine (RPV) / Dolutegravir (DTG) - EMEA-001750-PIP01-15-M03

ViiV Healthcare UK Limited; Unspecified Human immunodeficiency Virus (HIV) disease / Treatment of human immunodeficiency virus type 1 (HIV-1) infection

Day 60 opinion

Action: For adoption

Infectious Diseases

2.3.19. Tenofovir alafenamide / emtricitabine / bictegravir - EMEA-001766-PIP01-15-M02

Gilead Sciences International Ltd.; Treatment of human immunodeficiency virus (HIV) disease resulting in other conditions / for the treatment of adults and paediatrics aged less than 2 years weighing more than 4 kg infected with human immunodeficiency virus-1 (HIV-1) without any known mutations associated with resistance to the individual components

Day 60 opinion

Action: For adoption

Infectious Diseases

2.3.20. Hydrocortisone - EMEA-002305-PIP01-17-M01

LABORATOIRE AGUETTANT; Bronchopulmonary dysplasia.

Day 60 opinion

Action: For adoption

Neonatology - Paediatric Intensive Care

2.3.21. Cannabidiol - Orphan - EMEA-001964-PIP01-16-M02

GW Pharma (International) B.V.; Lennox Gastaut syndrome / Tuberous sclerosis complex /

Infantile spasms / Dravet syndrome / Treatment of seizures

Day 60 opinion

Action: For adoption

Neurology

2.3.22. Lacosamide - EMEA-000402-PIP03-17-M04

UCB Pharma S.A.; Treatment of generalised epilepsy and epileptic syndromes

Day 60 opinion

Action: For adoption

Neurology

2.3.23. Onasemnogene abeparvovec - Orphan - EMEA-002168-PIP01-17-M03

AveXis EU Limited; Treatment of spinal muscular atrophy / Treatment of spinal muscular atrophy Type 1

Day 60 opinion

Action: For adoption

Neurology

2.3.24. Ozanimod - EMEA-001710-PIP02-14-M05

Celgene Europe B.V.; Treatment of multiple sclerosis

Day 60 opinion

Action: For adoption

Neurology

2.3.25. Iodine (131-I) murine IgG1 monoclonal antibody against B7-H3 (131I-omburtamab) - Orphan - EMEA-002101-PIP02-18-M01

Y-mAbs Therapeutics A/S; Treatment of pediatric neuroblastoma patients with CNS relapse as evidenced by CNS/LM metastases

Day 60 opinion

Action: For adoption

Oncology

2.3.26. Ixazomib - Orphan - EMEA-001410-PIP02-17-M03

Takeda Pharm A/S; Treatment of lymphoid malignancies (excluding multiple myeloma) / Treatment of multiple myeloma (MM) / Treatment of adult patients with newly diagnosed

multiple myeloma (NDMM) / Treatment of paediatric patients diagnosed with relapsed precursor B-ALL or T-ALL

Day 60 opinion

Action: For adoption

Oncology

2.3.27. Midostaurin - Orphan - EMEA-000780-PIP01-09-M05

Novartis Europharm Limited; Malignant mastocystosis / Mast cell leukemia / Acute myeloid leukemia / Treatment of pediatric patients with FLT3 mutated AML, newly diagnosed

Day 60 opinion

Action: For adoption

Oncology

2.3.28. Quizartinib - Orphan - EMEA-001821-PIP01-15-M04

Daiichi Sankyo Europe GmbH; Treatment of acute myeloid leukaemia / For the treatment of paediatric patients aged from 1 month to less than 18 years of age with newly diagnosed AML with FLT3-ITD mutations

Day 60 opinion

Action: For adoption

Oncology

2.3.29. Venetoclax - Orphan - EMEA-002018-PIP02-16-M03

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, in patients from 1 month to 18 years of age

Day 60 opinion

Action: For adoption

Oncology / Haematology-Hemostaseology

2.3.30. Lenadogene nolparvovec - Orphan - EMEA-001992-PIP02-16-M01

GenSight-Biologics; Leber hereditary optic neuropathy (LHON)

Day 60 opinion

Action: For adoption

Ophthalmology

2.3.31. Andexanet alfa - EMEA-001902-PIP01-15-M04

Portola Netherlands B.V.; Prevention of factor Xa inhibitor associated haemorrhage / Treatment of factor Xa inhibitor associated haemorrhage / For the reversal of anticoagulation due to direct and indirect factor Xa inhibitors in patients requiring urgent surgery / For the reversal of anticoagulation due to direct and indirect factor Xa inhibitors in patients experiencing an acute major bleeding event

Day 60 opinion

Action: For adoption

Other

2.3.32. Vilanterol / fluticasone furoate - EMEA-000431-PIP01-08-M11

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

Day 60 opinion

Action: For adoption

Pneumology - Allergology

2.3.33. Synthetic double-stranded siRNA oligonucleotide directed against lactate dehydrogenase A mRNA and containing four modified nucleosides which form a ligand cluster of four N-acetylgalactosamine residues - Orphan - EMEA-002493-PIP01-18-M01

Dicerna Ireland Limited; Primary hyperoxaluria

Day 60 opinion

Action: For adoption

Uro-nephrology

2.3.34. Influenza virus surface antigens (haemagglutinin and neuraminidase) of strain B (Victoria lineage) / Influenza virus surface antigens (haemagglutinin and neuraminidase) of strain B (Yamagata lineage) / Influenza virus surface antigens (haemagglutinin and neuraminidase) of strain A (H3N2) / Influenza virus surface antigens (haemagglutinin and neuraminidase) of strain A (H1N1) - EMEA-001715-PIP01-14-M04

Seqirus Netherlands B.V.; Influenza / Prevention of influenza

Day 60 opinion

Action: For adoption

Vaccines

2.4. Opinions on Re-examinations

No items

2.5. Opinions on Review of Granted Waivers

No items

2.6. Finalisation and adoption of opinions

No items

2.7. Partial Compliance Checks completed by EMA

For the following partial compliance checks, no need to refer them to PDCO Committee for discussion, were identified by the PME coordinator and PDCO Rapporteur. The PDCO has been informed in writing.

2.7.1. Pegcetacoplan - EMEA-C1-002600-PIP01-19

Apellis Ireland Limited; Paroxysmal nocturnal haemoglobinuria

Day 30 letter

Action: For information

Haematology-Hemostaseology

2.7.2. Etrolizumab - EMEA-C1-001434-PIP01-13-M03

Roche Registration GmbH; Treatment of Crohn's disease

Day 30 letter

Action: For information

Gastroenterology-Hepatology

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. Glycopyrronium bromide - EMEA-002383-PIP01-18

Treatment of primary axillary hyperhidrosis

Day 90 discussion

Action: For discussion

Dermatology

3.1.2. Ruxolitinib - EMEA-002618-PIP01-19

Vitiligo

Day 90 discussion

Action: For discussion

Dermatology

3.1.3. 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 90 discussion

Action: For discussion

Haematology-Hemostaseology

3.1.4. Danicopan - Orphan - EMEA-002310-PIP01-17

Alexion Europe SAS; Treatment of paroxysmal nocturnal haemoglobinuria

Day 90 discussion

Action: For discussion

Haematology-Hemostaseology

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

Treatment of hereditary angioedema

Day 90 discussion

Action: For discussion

Haematology-Hemostaseology

3.1.6. 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 90 discussion

Action: For discussion

Infectious Diseases

3.1.7. Mixture of 2 synthetic double-stranded N-acetyl-galactosamine conjugated siRNA oligonucleotides that are directed against the hepatitis B virus - EMEA-002694-PIP01-19

Treatment of chronic viral hepatitis B / Treatment of chronic hepatitis B infection

Day 90 discussion

Action: For discussion

Infectious Diseases

3.1.8. EMEA-002693-PIP01-19

Treatment of chronic viral hepatitis B / Treatment of chronic hepatitis B infection

Day 90 discussion

Action: For discussion

Infectious Diseases

3.1.9. The whole range of unmanipulated autologous mononuclear cells derived from human umbilical cord blood (Hau-UCB-mnc) - Orphan - EMEA-001799-PIP03-19

BrainRepair UG (haftungsbeschränkt); Periventricular leukomalacia (PVL)

Day 90 discussion

Action: For discussion

Neonatology - Paediatric Intensive Care

3.1.10. rAAVrh74.MHCK7.microdystrophin - Orphan - EMEA-002677-PIP01-19

Sarepta Therapeutics Ireland; Duchenne muscular dystrophy

Day 90 discussion

Action: For discussion

Neurology

3.1.11. 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 90 discussion

Action: For discussion

Oncology

3.1.12. 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 90 discussion

Action: For discussion

Oncology

3.1.13. 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 90 discussion

Action: For discussion

Ophthalmology

3.1.14. 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 90 discussion

Action: For discussion

Other

3.1.15. EMEA-002705-PIP02-19

IgA Nephropathy

Day 90 discussion

Action: For discussion

Other

3.1.16. Finerenone - EMEA-001623-PIP02-20

Treatment of heart failure / Treatment of paediatric patients with heart failure and reduced ejection fraction

Day 60 discussion

Action: For discussion

Cardiovascular Diseases

3.1.17. EMEA-002778-PIP01-20

Diagnosis by evaluation of any known or suspected clinical condition with contrast enhanced magnetic resonance imaging

Day 60 discussion

Action: For discussion

Diagnostic

3.1.18. A self-complementary adeno-associated virus [AAV] serotype 8 virus particle encoding the human ornithine transcarbamylase [OTC] gene sequence - Orphan - EMEA-002830-PIP01-20

Ultragenyx Germany GmbH; Late-onset OTC deficiency

Day 60 discussion

Action: For discussion

Endocrinology-Gynaecology-Fertility-Metabolism

3.1.19. Recombinant adeno-associated viral vector serotype 9 containing the human N-α-acetylglucosaminidase gene - Orphan - EMEA-002764-PIP01-20

Abeona Therapeutics Inc.; Treatment of mucopolysaccharidosis type IIIB (Sanfilippo B syndrome) in children from birth to less than 18 years of age

Day 60 discussion

Action: For discussion

Endocrinology-Gynaecology-Fertility-Metabolism

3.1.20. Firsocostat / cilofexor - EMEA-002828-PIP01-20

Other specified inflammatory liver diseases (non-alcoholic steatohepatitis (NASH) / Treatment of non-alcoholic Steatohepatitis (NASH) with fibrosis in paediatric subjects, 8 to < 18 years of age

Day 60 discussion

Action: For discussion

Gastroenterology-Hepatology

3.1.21. Icosabutate - EMEA-002816-PIP01-20

Nonalcoholic fatty liver disease (NAFLD) with fibrosis or NASH

Day 60 discussion

Action: For discussion

Gastroenterology-Hepatology

3.1.22. Rozibafusp alfa - EMEA-002815-PIP01-20

Systemic lupus erythematosus

Day 60 discussion

Action: For discussion

Immunology-Rheumatology-Transplantation

3.1.23. Telitacicept - EMEA-002824-PIP01-20

Treatment of systemic lupus erythematosus

Day 60 discussion

Action: For discussion

Immunology-Rheumatology-Transplantation

3.1.24. 5'-cEtG-sp-cEt5MeU-sp-cEt5MeU-sp-dT-sp-dA-sp-dT-sp-dT-sp-dA-sp-dT-sp-dA-sp-dG-sp-dG-sp-dG-sp-cEt5MeC-sp-cEt5MeU-sp-cEt5MeU-3' - Orphan - EMEA-002609-PIP01-19

Dynacure S.A.S; Centronuclear myopathies

Day 60 discussion

Action: For discussion

Neurology

3.1.25. Surufatinib - EMEA-002750-PIP01-19

Treatment of all conditions included in the category of malignant neoplasms (except central nervous system tumours, haematopoietic and lymphoid tissue neoplasms)

Day 60 discussion

Action: For discussion

Oncology

3.1.26. Linear single strand of deoxyribonucleic acid (encoding human retinitis pigmentosa GTPase regulator [RPGR]) packaged in a recombinant adeno-associated virus protein capsid of serotype 5 (AAV2/5-hRKp.RPGR) - Orphan - EMEA-002827-PIP01-20

MeiraGTx UK II Ltd; Retinitis pigmentosa / RPGR mutation-associated X-linked retinitis pigmentosa

Day 60 discussion

Action: For discussion

Ophthalmology

3.1.27. Zilucoplan - EMEA-002747-PIP01-20

Treatment of myasthenia gravis

Day 60 discussion

Action: For discussion

Other / Neurology

3.1.28. Sparsentan - EMEA-001984-PIP03-20

Treatment of IgA Nephropathy (IgAN)

Day 60 discussion

Action: For discussion

Uro-nephrology

3.1.29. Live attenuated poliovirus type 3 / Live attenuated poliovirus type 1 - EMEA-002799-PIP01-20

Acute poliomyelitis

Day 60 discussion

Action: For discussion

Vaccines

3.1.30. Colchicine - EMEA-002837-PIP01-20

Secondary prevention of atherothrombotic events in patients with coronary artery disease by reducing local plaque inflammation and subsequent plaque erosion and/or rupture

Day 30 discussion

Action: For discussion

Cardiovascular Diseases

3.1.31. Fenofibrate / ezetimibe / eravastatin - EMEA-002835-PIP01-20

Treatment of mixed hyperlipidaemia

Day 30 discussion

Action: For discussion

Cardiovascular Diseases

3.1.32. Dupilumab - EMEA-001501-PIP06-20

Prurigo nodularis

Day 30 discussion

Action: For discussion

Dermatology

3.1.33. Dupilumab - EMEA-001501-PIP07-20

Treatment of chronic spontaneous urticaria

Day 30 discussion

Action: For discussion

Dermatology

3.1.34. LM-030 - Orphan - EMEA-002770-PIP02-20

LifeMax Laboratories, Inc.; Netherton syndrome

Day 30 discussion

Action: For discussion

Dermatology

3.1.35. Adeno-associated virus serotype 5- (AAV5-) based vector that contains the human phenylalanine hydroxylase (hPAH) gene - Orphan - EMEA-002833-PIP01-20

BioMarin International Limited; Treatment of phenylalanine hydroxylase deficiency / Treatment of patients with phenylketonuria

Day 30 discussion

Action: For discussion

Endocrinology-Gynaecology-Fertility-Metabolism

3.1.36. Dasiglucagon - Orphan - EMEA-002233-PIP02-20

Zealand Pharma; Congenital Hyperinsulinism

Day 30 discussion

Action: For discussion

Endocrinology-Gynaecology-Fertility-Metabolism

3.1.37. Hydroxypropyl-β-cyclodextrin (HPβCD) - Orphan - EMEA-002839-PIP01-20

Cyclo Therapeutics Inc; Niemann Pick disease type C / Treatment of Niemann Pick type C1 disease in children, adolesecents and adults

Day 30 discussion

Action: For discussion

Endocrinology-Gynaecology-Fertility-Metabolism

3.1.38. Liposomal annamycin - EMEA-002810-PIP01-20

Treatment of acute myeloid leukaemia / Treatment of relapsed acute myeloid leukaemia

Day 30 discussion

Action: For discussion

Haematology-Hemostaseology

3.1.39. EMEA-002350-PIP02-20

Treatment of chronic idiopathic arthritis (including rheumatoid arthritis, psoriatic arthritis, ankylosing spondylitis and juvenile idiopathic arthritis) / Treatment of juvenile psoriatic arthritis (JPsA) in paediatric patients 6 years of age and older

Day 30 discussion

Action: For discussion

Immunology-Rheumatology-Transplantation

3.1.40. Fenebrutinib - EMEA-002349-PIP02-20

Chronic idiopathic arthritis (including RA, axial spondyloarthritis, PsA, and JIA)

Day 30 discussion

Action: For discussion

Immunology-Rheumatology-Transplantation

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

Pre-procedure prevention of acute hereditary angioedema (HAE) / Treatment of hereditary angioedema (HAE)

Day 30 discussion

Action: For discussion

Immunology-Rheumatology-Transplantation

3.1.42. Bimekizumab - EMEA-002189-PIP04-20

Treatment of hidradenitis suppurativa / Treatment of moderate to severe hidradenitis suppurativa in adolescents from 12 years of age

Day 30 discussion

Action: For discussion

Immunology-Rheumatology-Transplantation / Dermatology

3.1.43. Risankizumab - EMEA-001776-PIP05-20

Hidradenitis suppurativa

Day 30 discussion

Action: For discussion

Immunology-Rheumatology-Transplantation / Dermatology

3.1.44. 2'-O-(2-methoxyethyl) modified antisense oligonucleotide targeting glial fibrillary acidic protein pre-mRNA - Orphan - EMEA-002822-PIP01-20

Ionis Pharmaceuticals; Alexander disease

Day 30 discussion

Action: For discussion

Neurology

3.1.45. Autologous T lymphocyte-enriched population of cells transduced with a lentiviral vector encoding a chimeric antigen receptor targeting human B cell maturation antigen with 4-1BB and CD3-zeta intracellular signalling domains - EMEA-002823-PIP01-20

Treatment of multiple myeloma / Treatment of patients with relapsed and/or refractory multiple myeloma (RRMM) who have received at least one proteasome inhibitor (PI), one immunomodulatory agent (IMiD), and one anti-CD38 monoclonal antibody (mAb). The patients shall have received at least 3 prior lines of therapy for MM

Day 30 discussion

Action: For discussion

Oncology

3.1.46. Dabrafenib - EMEA-001147-PIP02-20

Treatment of glioma / Treatment of paediatric patients with glioma with a BRAF V600 mutation in combination with trametinib

Day 30 discussion

Action: For discussion

Oncology

3.1.47. Erdafitinib - EMEA-002042-PIP02-20

Treatment of all conditions included in the category of malignant neoplasms (except 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 30 discussion

Action: For discussion

Oncology

3.1.48. Imetelstat - Orphan - EMEA-001910-PIP03-20

Geron Corporation; Treatment of acute myeloid leukaemia (AML) / Treatment of myelodysplastic syndromes (MDS), including juvenile myelomonocytic leukaemia (JMML) / Treatment of paediatric patients with relapsed or refractory AML or MDS, including JMML, from 1 year to less than 18 years of age

Day 30 discussion

Action: For discussion

Oncology

3.1.49. Sacituzumab govitecan - EMEA-002645-PIP02-20

Treatment of urothelial carcinoma

Day 30 discussion

Action: For discussion

Oncology

3.1.50. Tiragolumab - EMEA-002721-PIP02-20

Treatment of cervical cancer

Day 30 discussion

Action: For discussion

Oncology

3.1.51. Tiragolumab - EMEA-002721-PIP03-20

Treatment of oesophageal carcinoma

Day 30 discussion

Action: For discussion

Oncology

3.1.52. Trametinib - EMEA-001177-PIP02-20

Treatment of glioma / Treatment of paediatric patients with glioma with a BRAF V600 mutation in combination with dabrafenib

Day 30 discussion

Action: For discussion

Oncology

3.1.53. Alpha-R-lipoic acid choline ester tosilate - EMEA-002811-PIP01-20

Treatment of presbyopia

Day 30 discussion

Action: For discussion

Ophthalmology

3.1.54. Brolucizumab - EMEA-002691-PIP02-20

Diabetic retinopathy

Day 30 discussion

Action: For discussion

Ophthalmology

3.1.55. Recombinant humanised monoclonal antibody (IgG1, Kappa) to IL-5 - EMEA-002836-PIP01-20

Treatment of asthma / Add-on treatment for patients adolescents aged 12 years and older) with severe asthma and an eosinophilic phenotype

Day 30 discussion

Action: For discussion

Pneumology - Allergology

3.1.56. Conditionally replication-defective human cytomegalovirus (CMV) - EMEA-002838-PIP01-20

Congenital CMV infection and disease / Prevention of congenital CMV infection and disease

Day 30 discussion

Action: For discussion

Vaccines

3.1.57. Respiratory Syncytial Virus (RSV) PreF3 recombinant fusion protein - EMEA-002821-PIP01-20

Prevention of RSV- associated lower respiratory tract illness through maternal immunization / Active immunization of pregnant women during the second and third trimester of pregnancy to prevent respiratory syncytial virus (RSV) -associated lower respiratory tract illness (LRTI) in infants by transfer of maternal antibodies

Day 30 discussion

Action: For discussion

Vaccines

3.1.58. EMEA-000309-PIP05-20

Treatment of coronavirus disease 19 (COVID19)

D30 discussion

Action: For discussion

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. Ixekizumab - EMEA-C3-001050-PIP01-10-M05

Eli Lilly and Company Limited; Treatment of psoriasis

Day 30 discussion

Action: For discussion

Dermatology

3.2.2. Avalglucosidase alfa - EMEA-C1-001945-PIP01-16-M02

Genzyme Europe B.V.; Treatment of Pompe disease

Day 30 discussion

Action: For discussion

Endocrinology-Gynaecology-Fertility-Metabolism

3.2.3. Evinacumab - EMEA-C1-002298-PIP01-17-M01

Regeneron Ireland DAC; Treatment of elevated cholesterol

Day 30 discussion

Action: For discussion

Endocrinology-Gynaecology-Fertility-Metabolism / Cardiovascular Diseases

3.2.4. Maralixibat chloride - EMEA-C1-001475-PIP03-17-M02

Mirum Pharmaceuticals; Treatment of progressive familial intrahepatic cholestasis

Day 30 discussion

Action: For discussion

Gastroenterology-Hepatology

3.2.5. Pegcetacoplan - EMEA-C1-002600-PIP01-19

Apellis Ireland Limited; Paroxysmal nocturnal haemoglobinuria

Day 30 discussion

Action: For discussion

Haematology-Hemostaseology

3.2.6. Elivaldogene autotemcel - EMEA-C-001244-PIP01-11-M02

bluebird bio (Netherlands) B.V.; Treatment of adrenoleukodystrophy

Day 30 discussion

Action: For discussion

Neurology

3.2.7. Ad26.ZEBOV (recombinant, replication-incompetent) - EMEA-C2-002307-PIP01-17-M01

Janssen-Cilag International NV; Prevention of Ebola virus disease

Day 30 discussion

Action: For discussion

Vaccines

3.2.8. MVA-BN-Filo (recombinant, non-replicating) - EMEA-C2-002308-PIP01-17-M01

Janssen-Cilag International NV; Prevention of Ebola virus disease

Day 30 discussion

Action: For discussion

Vaccines

3.3. Discussions on Modification of an Agreed Paediatric Investigation Plan

3.3.1. Etripamil - EMEA-002303-PIP01-17-M02

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

Day 30 discussion

Action: For discussion

Cardiovascular Diseases

3.3.2. Edoxaban tosylate - EMEA-000788-PIP02-11-M10

Daiichi Sankyo Europe GmbH; Prevention of arterial thromboembolism / Prevention of venous thromboembolism /Treatment of venous thromboembolism / Prevention of arterial thromboembolism in paediatric cardiac patients at risk of thrombotic events / Acute treatment & secondary prevention of symptomatic recurrent venous thrombotic events (VTE) in paediatric patients at risk

Day 30 discussion

Action: For discussion

Cardiovascular Diseases / Haematology-Hemostaseology

3.3.3. Birch bark extract - Orphan - EMEA-001299-PIP03-17-M01

Amryt Research Limited; Treatment of epidermolysis bullosa

Day 30 discussion

Action: For discussion

Dermatology

3.3.4. Velmanase alfa - Orphan - EMEA-001056-PIP02-12-M01

Chiesi Farmaceutici S.p.A.; Treatment of alpha-mannosidosis

Day 30 discussion

Action: For discussion

Endocrinology-Gynaecology-Fertility-Metabolism

3.3.5. Ozanimod hydrochloride - EMEA-001710-PIP03-17-M02

Celgene Europe B.V.; Treatment of ulcerative colitis / Treatment of moderate to severely active ulcerative colitis

Day 30 discussion

Action: For discussion

Gastroenterology-Hepatology

3.3.6. Pegylated-fibroblast growth factor 21 - EMEA-002448-PIP01-18-M01

Bristol-Myers Squibb International Corporation; Treatment of non-alcoholic steatohepatitis (NASH) / Treatment of NASH with moderate to severe liver fibrosis

Day 30 discussion

Action: For discussion

Gastroenterology-Hepatology

3.3.7. Mepolizumab - Orphan - EMEA-000069-PIP01-07-M07

GSK Trading Services Limited; Treatment of hypereosinophilic syndrome (HES)

Day 30 discussion

Action: For discussion

Haematology-Hemostaseology

3.3.8. Vadadustat - EMEA-001944-PIP01-16-M02

Akebia Therapeutics, Inc.; Treatment of anaemia due to chronic disorders / Treatment of anaemia secondary to chronic kidney disease

Day 30 discussion

Action: For discussion

Haematology-Hemostaseology

3.3.9. Zanamivir - EMEA-001318-PIP01-12-M04

GlaxoSmithKline Trading Services Limited; Treatment of influenza / Prevention of influenza / Treatment of influenza A and B virus infection / Prevention of influenza A and B virus infection

Day 30 discussion

Action: For discussion

Infectious Diseases

3.3.10. Brivaracetam - Orphan - EMEA-000332-PIP01-08-M15

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

Day 30 discussion

Action: For discussion

Neurology

3.3.11. Eculizumab - Orphan - EMEA-000876-PIP05-15-M04

Alexion Europe SAS; Myasthenia gravis / Treatment of refractory acetylcholine receptor antibody (AChR-Ab) - positive generalised myasthenia gravis

Day 30 discussion

Action: For discussion

Neurology

3.3.12. Copanlisib dihydrochloride - Orphan - EMEA-001757-PIP02-15-M02

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

Day 30 discussion

Action: For discussion

Oncology

3.3.13. Dabrafenib - EMEA-001147-PIP01-11-M07

Novartis Europharm Limited; Treatment of solid malignant tumours (excluding melanoma and glioma) / Treatment of melanoma / Treatment of paediatric patients with solid malignant tumours containing BRAF V600 mutations / Treatment of adolescent patients with melanoma with a BRAF V600 mutation

Day 30 discussion

Action: For discussion

Oncology

3.3.14. Daratumumab - Orphan - EMEA-002152-PIP01-17-M02

Janssen-Cilag International NV; Treatment of lymphoid malignancies (except mature B-cell neoplasms) / Treatment of paediatric patients from birth to less than 18 years of age with a lymphoid malignancy (except mature B-cell neoplasms)

Day 30 discussion

Action: For discussion

Oncology

3.3.15. Lenvatinib - EMEA-001119-PIP02-12-M07

Eisai GmbH; Treatment of osteosarcoma / Treatment of papillary thyroid carcinoma /

Treatment of follicular carcinoma / Treatment of refractory or relapsed osteosarcoma in children and adolescents / Treatment of progressive, radioiodine-refractory differentiated thyroid cancer in children and adolescents

Day 30 discussion

Action: For discussion

Oncology

3.3.16. Trametinib - EMEA-001177-PIP01-11-M06

Novartis Europharm Limited; Treatment of all conditions included in the category of malignant neoplasms (except melanoma, glioma, haematopoietic and lymphoid tissue) / Treatment of melanoma / Treatment of paediatric patients with a solid malignant tumour with known or expected RAS, RAF or MEK pathway activation / Treatment of adolescent patients with melanoma containing BRAF V600 activating mutations

Day 30 discussion

Action: For discussion

Oncology

3.3.17. Ivacaftor / tezacaftor / elexacaftor - Orphan - EMEA-002324-PIP01-17-M01

Vertex Pharmaceuticals (Ireland) Limited; Treatment of cystic fibrosis

Day 30 discussion

Action: For discussion

Other / Pneumology - Allergology

3.3.18. Dupilumab - EMEA-001501-PIP02-13-M06

sanofi-aventis recherche & développement; Treatment of asthma

Day 30 discussion

Action: For discussion

Pneumology - Allergology

3.3.19. Peanut Allergen Extract - EMEA-001481-PIP01-13-M04

DBV Technologies S.A.; Peanut allergy

Day 30 discussion

Action: For discussion

Pneumology - Allergology

3.3.20. Ravulizumab - Orphan - EMEA-001943-PIP01-16-M05

Alexion Europe SAS; Treatment of atypical haemolytic uremic syndrome

Day 30 discussion

Action: For discussion

Uro-nephrology / Haematology-Hemostaseology

3.3.21. Ravulizumab - Orphan - EMEA-002077-PIP01-16-M03

Alexion Europe SAS; Treatment of paroxysmal nocturnal haemoglobinuria

Day 30 discussion

Action: For discussion

Uro-nephrology / 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 letters of intent received for submission of applications with start of procedure 18 August 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

4.3.1. Nomination of expert to the Non-clinical Working Group (NcWG)

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. Selective estrogen receptor degrader - EMEA-06-2020

Sanofi-Aventis Recherche & Développement; The classes of androgen receptor modulator, of oestrogen receptor modulator, of growth and sex hormone as well as their releasing of inhibiting factors, and of sex hormone-metabolism modulator medicinal products for treatment of breast malignant neoplasms, prostate malignant neoplasms and neuroendocrine malignant neoplasm/ Treatment of estrogen receptor (ER)-positive, human epidermal growth factor receptor 2 (HER2)-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 item

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

No item

9.2. Coordination with EMA Scientific Committees or CMDh-v

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

Action: For information

9.2.2. Committee for Advanced Therapies (CAT)

Improving CAT-PDCO interactions

Action: For information

9.2.3. Committee on Herbal Medicinal Products (HMPC)

HMPC request for PDCO input

Action: For discussion

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

No item

9.5. Cooperation with International Regulators

9.5.1. Report from the Paediatric Cluster Teleconference

Action: For information

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

No item

9.7. PDCO work plan

No item

9.8. Planning and reporting

9.8.1. Strategic Review and Learning Meeting (SRLM) under the German Presidency to be held virtually on 22nd October 2020

Action: for information

10. Any other business

10.1.1. EMA Working Parties review

Action: For information

10.1.2. Covid-19 update

Action: For information

11. Breakout sessions

11.1.1. Paediatric oncology

Action: For discussion on Wednesday, 12:45 - 14:00

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/