



EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

18 June 2020
EMA/PDCO/302368/2020
Human Medicines Division

Paediatric Committee (PDCO)

Agenda for the meeting on 23-26 June 2020

Chair: Koenraad Norga – Vice-Chair: Sabine Scherer

23 June 2020, 13:00- 19:00, Virtual meeting

24 June 2020, 08:30- 19:00, Virtual meeting

25 June 2020, 08:30- 19:00, Virtual meeting

26 June 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 ongoing 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 June 2020. See June 2020 PDCO minutes (to be published post June 2020 PDCO meeting).

1.2. Adoption of agenda

PDCO agenda for 23-26 June 2020

1.3. Adoption of the minutes

PDCO minutes for 26-29 May 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. Lonapegsomatropin - EMEA-002692-PIP01-19

Growth hormone deficiency

Day 120 opinion

Action: For adoption

Endocrinology-Gynaecology-Fertility-Metabolism

2.1.2. Cilofexor - Orphan - EMEA-002554-PIP02-19

Gilead Sciences International Ltd.; Treatment of primary sclerosing cholangitis (PSC)

Day 120 opinion

Action: For adoption

Gastroenterology-Hepatology

2.1.3. Guselkumab - EMEA-001523-PIP05-19

Crohn's Disease / Treatment of Crohn's disease

Day 120 opinion

Action: For adoption

Gastroenterology-Hepatology

2.1.4. [Marstacimab - Orphan - EMEA-002285-PIP02-19](#)

Pfizer Europe MA EEIG; Treatment of congenital haemophilia B / Treatment of congenital haemophilia A / Prophylaxis of bleeding in haemophilia B / Prophylaxis of bleeding in haemophilia A

Day 120 opinion

Action: For adoption

Haematology-Hemostaseology

2.1.5. [Rilzabrutinib - Orphan - EMEA-002438-PIP02-19](#)

Principia Biopharma, Inc.; Immune thrombocytopenia / Treatment of immune thrombocytopenia

Day 120 opinion

Action: For adoption

Immunology-Rheumatology-Transplantation

2.1.6. [Ritonavir / darunavir - EMEA-002537-PIP02-19](#)

Treatment of human immunodeficiency virus (HIV-1) infection

Day 120 opinion

Action: For adoption

Infectious Diseases

2.1.7. [EMEA-002566-PIP01-19](#)

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

Day 120 opinion

Action: For adoption

Neurology

2.1.8. [Soticlestat - EMEA-002572-PIP02-19](#)

Dravet Syndrome, Lennox-Gastaut Syndrome / Treatment of seizures associated with Dravet Syndrome / Treatment of seizures associated with Lennox-Gastaut syndrome

Day 120 opinion

Action: For adoption

Neurology

2.1.9. Orphan - EMEA-002571-PIP01-19

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

Day 120 opinion

Action: For adoption

Other

2.1.10. Fasinumab - EMEA-002059-PIP02-19

Chronic pain / Chronic musculoskeletal pain / Chronic non-musculoskeletal pain / Treatment of chronic cancer pain in a palliative care setting / Treatment of moderate to severe chronic pain associated with osteoarthritis (OA) of the knee or hip in patients who achieve an inadequate response to, or are intolerant to currently available analgesics (adults only)

Day 120 opinion

Action: For adoption

Pain

2.1.11. Ezetimibe / pravastatin - EMEA-002805-PIP01-20

Hypercholesterolaemia / Cardiovascular events

Day 60 opinion

Action: For adoption

Cardiovascular Diseases

2.1.12. Sacubitril / valsartan - EMEA-000316-PIP03-20

Prevention of heart failure

Day 60 opinion

Action: For adoption

Cardiovascular Diseases

2.1.13. Benralizumab - EMEA-001214-PIP06-20

Bullous Pemphigoid

Day 60 opinion

Action: For adoption

Dermatology

[2.1.14. Dupilumab - EMEA-001501-PIP05-20](#)

Treatment of Bullous Pemphigoid

Day 60 opinion

Action: For adoption

Dermatology

[2.1.15. Elafibranor - Orphan - EMEA-001857-PIP02-20](#)

Genfit SA; Treatment of primary biliary cholangitis

Day 60 opinion

Action: For adoption

Gastroenterology-Hepatology

[2.1.16. Balixafortide - EMEA-002718-PIP02-20](#)

Day 60 opinion

Action: For adoption

Oncology

[2.1.17. EMEA-002655-PIP02-20](#)

Treatment of prostate cancer

Day 60 opinion

Action: For adoption

Oncology

[2.1.18. Nivolumab - EMEA-001407-PIP03-20](#)

Treatment of all conditions in the category of malignant neoplasms (except haematopoietic and lymphoid tissue other than Hodgkin lymphoma)

Day 60 opinion

Action: For adoption

Oncology

[2.1.19. Opportuzumab monatox - EMEA-002797-PIP01-20](#)

Treatment, including prevention of recurrence, of urothelial carcinoma

Day 60 opinion

Action: For adoption

Oncology

2.1.20. Sasanlimab - EMEA-002777-PIP01-20

Non-muscle invasive bladder cancer (NMIBC)

Day 60 opinion

Action: For adoption

Oncology

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. Eptinezumab - EMEA-C1-002243-PIP01-17

Prevention of migraine headaches

Day 60 letter

Action: For adoption

Neurology

2.3. Opinions on Modification of an Agreed Paediatric Investigation Plan

2.3.1. Crisaborole - EMEA-002065-PIP01-16-M03

Pfizer Europe MA EEIG; Mild to moderate atopic dermatitis

Day 60 opinion

Action: For adoption

Dermatology

2.3.2. Efglenatide - EMEA-001903-PIP01-15-M01

Sanofi-aventis recherche et développement; Type 2 diabetes mellitus

Day 60 opinion

Action: For adoption

Endocrinology-Gynaecology-Fertility-Metabolism

2.3.3. Glucagon - EMEA-001657-PIP01-14-M01

Eli Lilly and Company; Treatment of severe hypoglycaemia

Day 60 opinion

Action: For adoption

Endocrinology-Gynaecology-Fertility-Metabolism

2.3.4. Ladarixin - EMEA-002642-PIP01-19-M01

Dompé farmaceutici S.p.A; Treatment of type 1 diabetes / Treatment of new-onset type 1 diabetes mellitus

Day 60 opinion

Action: For adoption

Endocrinology-Gynaecology-Fertility-Metabolism

2.3.5. Pegvaliase - Orphan - EMEA-001951-PIP01-16-M01

BioMarin International Limited; Treatment of hyperphenylalaninaemia in paediatric patients with phenylketonuria

Day 60 opinion

Action: For adoption

Endocrinology-Gynaecology-Fertility-Metabolism

2.3.6. Recombinant human glutamic acid decarboxylase - EMEA-000609-PIP01-09-M02

Diamyd Medical AB; Treatment of type 1 diabetes / Treatment of type 1 diabetes

Day 60 opinion

Action: For adoption

Endocrinology-Gynaecology-Fertility-Metabolism

2.3.7. Ozanimod hydrochloride - EMEA-001710-PIP04-17-M01

Celgene Europe B.V.; Treatment of Crohn's disease

Day 60 opinion

Action: For adoption

Gastroenterology-Hepatology

2.3.8. Ustekinumab - EMEA-000311-PIP04-13-M03

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

Day 60 opinion

Action: For adoption

Gastroenterology-Hepatology

2.3.9. [Ustekinumab - EMEA-000311-PIP05-17-M01](#)

Janssen-Cilag International NV; Treatment of ulcerative colitis

Day 60 opinion

Action: For adoption

Gastroenterology-Hepatology

2.3.10. [Synthetic double-stranded siRNA oligonucleotide directed against antithrombin mRNA and covalently linked to a ligand containing three N-acetylgalactosamine residues - Orphan - EMEA-001855-PIP01-15-M02](#)

Genzyme Europe B.V.; Treatment of Haemophilia B / Treatment of Haemophilia A / For routine prophylaxis to prevent or reduce the frequency of bleeding episodes in children aged ≥ 1 year with severe Haemophilia B, including patients who express neutralizing antibodies to exogenous factor IX substitution / For routine prophylaxis to prevent or reduce the frequency of bleeding episodes in children aged ≥ 1 year with severe haemophilia A, including patients who express neutralizing antibodies to exogenous factor VIII substitution

Day 60 opinion

Action: For adoption

Haematology-Hemostaseology

2.3.11. [Belatacept - EMEA-000157-PIP01-07-M05](#)

Bristol-Myers Squibb Pharma EEIG; Prevention of rejection of transplanted kidney / In combination with corticosteroids and a mycophenolic acid (MPA), for prophylaxis of graft rejection in pediatric patients at least 12 years of age and with a stable renal transplant for at least 6 months, who convert to a CNI-free maintenance immunosuppressive regimen

Day 60 opinion

Action: For adoption

Immunology-Rheumatology-Transplantation

2.3.12. [Brincidofovir - Orphan - EMEA-001904-PIP03-18-M01](#)

Chimerix IRL Limited; Treatment of smallpox

Day 60 opinion

Action: For adoption

Infectious Diseases

2.3.13. Ceftobiprole medocartil sodium - EMEA-000205-PIP02-11-M04

Basilea Pharmaceutica International Ltd.; Bacterial pneumoniae / Pneumonia due to Streptococcus pneumoniae / Pneumonia due to Hemophilus influenzae / Treatment of nosocomial pneumonia / Treatment of community acquired pneumonia

Day 60 opinion

Action: For adoption

Infectious Diseases

2.3.14. Dasabuvir sodium - EMEA-001439-PIP01-13-M03

AbbVie Ltd; Treatment of chronic hepatitis C / Treatment of children and adolescents aged from 3 years to less than 18 years of age with genotype I chronic HCV infection and without liver decompensation, in combination with ombitasvir/paritaprevir/ritonavir

Day 60 opinion

Action: For adoption

Infectious Diseases

2.3.15. Relebactam monohydrate / cilastatin sodium / imipenem monohydrate - EMEA-001809-PIP01-15-M02

Merck Sharp & Dohme (Europe), Inc.; Treatment of Gram-negative bacterial infections / Treatment of hospital-acquired bacterial pneumonia (HABP) and ventilator-associated bacterial pneumonia (VABP), Treatment of serious bacterial infections including HABP, VABP, complicated intra-abdominal infections (cIAI) and complicated urinary tract infections (cUTI) caused by aerobic Gram-negative organisms in patients with limited treatment options

Day 60 opinion

Action: For adoption

Infectious Diseases

2.3.16. Rilpivirine (hydrochloride) - EMEA-000317-PIP01-08-M12

Janssen-Cilag International NV; Treatment of human immunodeficiency virus (HIV-1) infection / Rilpivirine is indicated in combination with other antiretroviral (ARV) medicinal products, for the treatment of human immunodeficiency virus (HIV-1) infection in ARV-naïve paediatric patients from 2 to less than 18 years with a baseline viral load below 100,000 HIV-1 RNA copies/mL.

Day 60 opinion

Action: For adoption

Infectious Diseases

2.3.17. [Ritonavir / paritaprevir / ombitasvir - EMEA-001440-PIP01-13-M03](#)

AbbVie Ltd; Chronic Hepatitis C (HCV) infection / Treatment of children and adolescents from ≥ 3 years to < 18 years of age with chronic HCV infection with compensated cirrhosis or without compensated cirrhosis in combination with other medicinal products

Day 60 opinion

Action: For adoption

Infectious Diseases

2.3.18. [Lasmiditan - EMEA-002166-PIP01-17-M04](#)

Eli Lilly and Company Limited; Migraine with and without aura

Day 60 opinion

Action: For adoption

Neurology

2.3.19. [Olenasufligene relduparvovec - Orphan - EMEA-002122-PIP02-17-M01](#)

LYSOGENE; Mucopolysaccharidosis type IIIA / Treatment of children with Sanfilippo type A syndrome

Day 60 opinion

Action: For adoption

Neurology

2.3.20. [Perampanel - EMEA-000467-PIP01-08-M14](#)

Eisai Europe Limited; Treatment of treatment-resistant epilepsies / Adjunctive therapy in patients with other paediatric epilepsies / Adjunctive therapy in patients with refractory partial onset seizures including secondarily generalised seizures

Day 60 opinion

Action: For adoption

Neurology

2.3.21. [Pitolisant - Orphan - EMEA-001176-PIP01-11-M05](#)

BIOPROJET PHARMA; Narcolepsy / Narcolepsy with or without cataplexy

Day 60 opinion

Action: For adoption

Neurology

2.3.22. [Concentrate of proteolytic enzyme enriched in bromelain - Orphan - EMEA-000142-PIP02-09-M09](#)

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

2.3.23. [Palonosetron / fosnetupitant - EMEA-001198-PIP03-17-M04](#)

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

Day 60 opinion

Action: For adoption

Other

2.3.24. [Dermatophagoides pteronyssinus / Dermatophagoides farinae - EMEA-001258-PIP01-11-M06](#)

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

Day 60 opinion

Action: For adoption

Pneumology - Allergology

2.3.25. [Glycopyrronium bromide / formoterol fumarate dihydrate / beclometasone dipropionate - EMEA-001875-PIP02-18-M02](#)

Chiesi Farmaceutici S.p.A.; Treatment of asthma

Day 60 opinion

Action: For adoption

Pneumology - Allergology

2.3.26. [Reslizumab - EMEA-001202-PIP02-13-M04](#)

Teva Pharmaceuticals Europe; Treatment of asthma / Add on treatment to reduce exacerbations, relieve symptoms and improve lung function in paediatric patients from 6 to less than 18 years of age with inadequately controlled severe asthma who have a blood eosinophil count greater than equal to 300 per microL

Day 60 opinion

Action: For adoption

2.3.27. Derivative of 4H-pyrazolo[3,4-d]pyrimidin-4-one - EMEA-001742-PIP01-14-M01

Boehringer Ingelheim International GmbH; Treatment of schizophrenia / Adjunctive therapy to antipsychotic treatment for the prevention of relapse in patients with schizophrenia 13 to <18 years of age

Day 60 opinion

Action: For adoption

Psychiatry

2.3.28. Ebola Zaire Vaccine (rVSVΔG-ZEBOV-GP, live) - EMEA-001786-PIP01-15-M01

Merck Sharp & Dohme (Europe), Inc.; Prevention of Ebola disease / Active immunization of individuals from 1 year of age to less than 18 years of age to protect against Ebola virus disease (EVD) caused by Zaire Ebola virus

Day 60 opinion

Action: For adoption

Vaccines

2.3.29. Purified Rabies virus, WISTAR PM/WI 38-1503-3M strain (inactivated) - EMEA-002234-PIP01-17-M01

Sanofi Pasteur; Prevention of rabies disease / treatment of exposure to rabies virus

Day 60 opinion

Action: For adoption

Vaccines

2.4. Opinions on Re-examinations

No item

2.5. Opinions on Review of Granted Waivers

No item

2.6. Finalisation and adoption of opinions

No item

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. [Asciminib - EMEA-C1-002347-PIP01-18](#)

Novartis Europharm Limited; Treatment of chronic myeloid leukaemia

Day 30 letter

Action: For information

Oncology / Haematology-Hemostaseology

2.7.2. [Abrocitinib - EMEA-C1-002312-PIP01-17-M01](#)

Pfizer Europe MA EEIG; Treatment of atopic dermatitis

Day 30 letter

Action: For information

Dermatology

2.7.3. [Vaxchora - EMEA-C1-001490-PIP01-13-M01](#)

Emergent Netherlands B.V.; Treatment of cholera

Day 30 letter

Action: For information

Vaccines

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. [Bilayer, engineered, collagen hydrogel-based skin graft composed of autologous keratinocytes and fibroblasts - Orphan - EMEA-002699-PIP01-19](#)

CUTISS AG; Treatment of burns

Day 90 discussion

Action: For discussion

Dermatology

3.1.2. 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 90 discussion

Action: For discussion

Endocrinology-Gynaecology-Fertility-Metabolism

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

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

Day 90 discussion

Action: For discussion

Endocrinology-Gynaecology-Fertility-Metabolism

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

Albireo AB; Biliary atresia

Day 90 discussion

Action: For discussion

Gastroenterology-Hepatology

3.1.5. Relamorelin - EMEA-002323-PIP02-19

Day 90 discussion

Action: For discussion

Gastroenterology-Hepatology

3.1.6. 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 90 discussion

Action: For discussion

Haematology-Hemostaseology

3.1.7. Efgartigimod alfa - Orphan - EMEA-002597-PIP02-19

Argenx bv; Treatment of immune thrombocytopenia

Day 90 discussion

Action: For discussion

Haematology-Hemostaseology

3.1.8. Mitapivat - EMEA-002684-PIP01-19

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

Day 90 discussion

Action: For discussion

Haematology-Hemostaseology

3.1.9. Pegfilgrastim - EMEA-002671-PIP01-19

Treatment of chemotherapy-induced 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 90 discussion

Action: For discussion

Haematology-Hemostaseology

3.1.10. 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 90 discussion

Action: For discussion

Haematology-Hemostaseology

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

Amivas LLC; Malaria

Day 90 discussion

Action: For discussion

Infectious Diseases

3.1.12. [Diroximel - EMEA-002685-PIP02-19](#)

Treatment of multiple sclerosis

Day 90 discussion

Action: For discussion

Neurology

3.1.13. [EMEA-002631-PIP01-19](#)

Treatment of acute myeloid leukaemia

Day 90 discussion

Action: For discussion

Oncology

3.1.14. [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 90 discussion

Action: For discussion

Oncology

3.1.15. [Emixustat - EMEA-002581-PIP01-19](#)

Stargardt disease

Day 90 discussion

Action: For discussion

Ophthalmology

3.1.16. [Alpelisib - EMEA-002016-PIP03-19](#)

PIK3CA related overgrowth spectrum (PROS)

Day 90 discussion

Action: For discussion

Other

3.1.17. [Anti-neonatal Fc receptor human monoclonal antibody - Orphan - EMEA-002559-PIP03-19](#)

Momenta Pharmaceuticals, Inc.; Autoimmune haemolytic anaemia

Day 90 discussion

Action: For discussion

Other

3.1.18. [Levonorgestrel - EMEA-002767-PIP01-20](#)

Prevention of pregnancy

Day 60 discussion

Action: For discussion

Endocrinology-Gynaecology-Fertility-Metabolism

3.1.19. [Triheptanoin - Orphan - EMEA-001920-PIP04-19](#)

Ultragenyx Germany GmbH; Long-Chain fatty acid oxidation disorders (LC-FAOD) /
Treatment of long-chain fatty acid oxidation disorders (LC-FAOD)

Day 60 discussion

Action: For discussion

Endocrinology-Gynaecology-Fertility-Metabolism

3.1.20. [EMEA-002773-PIP01-20](#)

Treatment of non-alcoholic steatohepatitis (NASH)

Day 60 discussion

Action: For discussion

Gastroenterology-Hepatology

3.1.21. [EMEA-002757-PIP01-19](#)

Treatment of ulcerative colitis

Day 60 discussion

Action: For discussion

Gastroenterology-Hepatology

3.1.22. [Crovalimab - EMEA-002709-PIP01-19](#)

Treatment of paroxysmal nocturnal hemoglobinuria (PNH)

Day 60 discussion

Action: For discussion

Haematology-Hemostaseology

3.1.23. Olinciguat - EMEA-002759-PIP01-19

Treatment of sickle cell disease (SCD)

Day 60 discussion

Action: For discussion

Haematology-Hemostaseology

3.1.24. Voxelotor - Orphan - EMEA-002356-PIP02-20

Synteract GmbH; Sickle cell disease

Day 60 discussion

Action: For discussion

Haematology-Hemostaseology

3.1.25. Tacrolimus - EMEA-001642-PIP02-20

Solid organ transplant rejection / Prophylaxis of transplant rejection in liver, kidney or heart allograft recipients (children aged from birth to less than 18 years) / Treatment of allograft rejection resistant to treatment with other immunosuppressive medical products in children aged from birth to less than 18 years

Day 60 discussion

Action: For discussion

Immunology-Rheumatology-Transplantation

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

Non-responsive coeliac disease (NRCD) with symptoms despite 12 months of following a gluten-free diet (GFD)

Day 60 discussion

Action: For discussion

Immunology-Rheumatology-Transplantation / Gastroenterology-Hepatology

3.1.27. EMEA-002740-PIP01-19

Treatment of human immunodeficiency virus (HIV-1) infection

Day 60 discussion

Action: For discussion

Infectious Diseases

3.1.28. Sulbactam / durlobactam - EMEA-002807-PIP01-20

Treatment of bacterial infections / Treatment of infections caused by Acinetobacter baumannii-calcoaceticus complex

Day 60 discussion

Action: For discussion

Infectious Diseases

3.1.29. Rimegepant - EMEA-002812-PIP01-20

Prevention of migraine

Day 60 discussion

Action: For discussion

Neurology

3.1.30. EMEA-002779-PIP01-20

Treatment of Canavan disease / Treatment of Canavan disease in patients from birth to less than 18 years of age

Day 60 discussion

Action: For discussion

Neurology

3.1.31. 2-(hydroxymethyl)-2-(methoxymethyl)-1-azabicyclo[2,2,2]octan-3-one - Orphan - EMEA-002621-PIP01-19

Aprea Therapeutics Inc; Treatment of malignant neoplasms of the haematopoietic and lymphoid tissue / Treatment of solid malignant tumours

Day 60 discussion

Action: For discussion

Oncology / Haematology-Hemostaseology

3.1.32. Benzimidazole - EMEA-002394-PIP02-20

Treatment of cystic fibrosis / To improve lung function and reduce pulmonary exacerbations for patients with cystic fibrosis (CF) in conjunction with standard therapies

Day 60 discussion

Action: For discussion

Pneumology - Allergology

3.1.33. Seltorexant - EMEA-002746-PIP01-20

Major depressive disorder (MDD)

Day 60 discussion

Action: For discussion

Psychiatry

3.1.34. Sparsentan - Orphan - EMEA-001984-PIP02-20

Retrophin Europe Ltd.; Treatment of focal segmental glomerular sclerosis (FSGS)

Day 60 discussion

Action: For discussion

Uro-nephrology

3.1.35. EMEA-002814-PIP01-20

Invasive disease caused by Neisseria meningitidis group A, B, C, W and Y from 2 months of age

Day 60 discussion

Action: For discussion

Vaccines

3.1.36. EMEA-002795-PIP01-20

Prevention of RSV-associated medically attended lower respiratory tract illness (MA-LRTI) and/or RSV-associated severe MA LRTI in neonates and infants by 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 60 discussion

Action: For discussion

Vaccines

3.1.37. EMEA-002771-PIP01-20

Prevention of influenza disease

Day 60 discussion

Action: For discussion

Vaccines / Infectious Diseases

3.1.38. EMEA-002735-PIP02-20

Heart transplantation

Day 30 discussion

Action: For discussion

Cardiovascular Diseases

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

Prevention of cardiovascular events

Day 30 discussion

Action: For discussion

Cardiovascular Diseases

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

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

Day 30 discussion

Action: For discussion

Cardiovascular Diseases

3.1.41. Finerenone - EMEA-001623-PIP02-20

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

Day 30 discussion

Action: For discussion

Cardiovascular Diseases

3.1.42. EMEA-002778-PIP01-20

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

Day 30 discussion

Action: For discussion

Diagnostic

3.1.43. [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 30 discussion

Action: For discussion

Endocrinology-Gynaecology-Fertility-Metabolism

3.1.44. [Recombinant adeno-associated viral vector serotype 9 containing the human N- \$\alpha\$ -acetylglucosaminidase gene - Orphan - EMEA-002764-PIP01-20](#)

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

Day 30 discussion

Action: For discussion

Endocrinology-Gynaecology-Fertility-Metabolism

3.1.45. [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 30 discussion

Action: For discussion

Gastroenterology-Hepatology

3.1.46. [Icosabutate - EMEA-002816-PIP01-20](#)

Non-alcoholic fatty liver disease (NAFLD) with fibrosis or NASH

Day 30 discussion

Action: For discussion

Gastroenterology-Hepatology

3.1.47. [Linerixibat - EMEA-002800-PIP01-20](#)

Treatment of primary biliary cholangitis

Day 30 discussion

Action: For discussion

Gastroenterology-Hepatology

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

Systemic lupus erythematosus

Day 30 discussion

Action: For discussion

Immunology-Rheumatology-Transplantation

3.1.49. Telitacicept - EMEA-002824-PIP01-20

Treatment of systemic lupus erythematosus

Day 30 discussion

Action: For discussion

Immunology-Rheumatology-Transplantation

3.1.50. EMEA-002825-PIP01-20

Sicca syndrome (Sjögren's)

Day 30 discussion

Action: For discussion

Immunology-Rheumatology-Transplantation

3.1.51. 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 30 discussion

Action: For discussion

Neurology

3.1.52. Fruquintinib - EMEA-002784-PIP01-20

Treatment of colorectal carcinoma

Day 30 discussion

Action: For discussion

Oncology

3.1.53. 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 30 discussion

Action: For discussion

Oncology

3.1.54. EMEA-002798-PIP01-20

Treatment of squamous carcinoma of the anal canal (SCAC)

Day 30 discussion

Action: For discussion

Oncology

3.1.55. 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 30 discussion

Action: For discussion

Oncology

3.1.56. Faricimab - EMEA-002817-PIP03-20

Diabetic retinopathy

Day 30 discussion

Action: For discussion

Ophthalmology

3.1.57. Faricimab - EMEA-002817-PIP04-20

Retinal vein occlusion

Day 30 discussion

Action: For discussion

Ophthalmology

3.1.58. 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 30 discussion

Action: For discussion

Ophthalmology

3.1.59. [Ranibizumab - EMEA-002832-PIP01-20](#)

Diabetic Retinopathy

Day 30 discussion

Action: For discussion

Ophthalmology

3.1.60. [Dronabinol - EMEA-000643-PIP02-20](#)

Treatment of spasticity

Day 30 discussion

Action: For discussion

Other / Neurology

3.1.61. [Zilucoplan - EMEA-002747-PIP01-20](#)

Treatment of myasthenia gravis

Day 30 discussion

Action: For discussion

Other / Neurology

3.1.62. [Tezepelumab - EMEA-001613-PIP02-20](#)

Chronic rhinosinusitis with nasal polyps

Day 30 discussion

Action: For discussion

Oto-rhino-laryngology

3.1.63. [Sparsentan - EMEA-001984-PIP03-20](#)

Treatment of IgA Nephropathy (IgAN)

Day 30 discussion

Action: For discussion

Uro-nephrology

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

Acute poliomyelitis

Day 30 discussion

Action: For discussion

Vaccines

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

Boehringer Ingelheim International GmbH; Prevention of dabigatran associated haemorrhage

Day 30 discussion

Action: For discussion

Cardiovascular Diseases / Haematology-Hemostaseology

3.2.2. Empagliflozin - EMEA-C1-000828-PIP01-09

Boehringer Ingelheim International GmbH; Treatment of type 2 diabetes mellitus

Day 30 discussion

Action: For discussion

Endocrinology-Gynaecology-Fertility-Metabolism

3.2.3. Semaglutide - EMEA-C1-001441-PIP03-17-M01

Novo Nordisk A/S; Treatment of obesity

Day 30 discussion

Action: For discussion

Endocrinology-Gynaecology-Fertility-Metabolism

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

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

Day 30 discussion

Action: For discussion

Haematology-Hemostaseology

3.2.5. [Risdiplam - EMEA-C1-002070-PIP01-16-M04](#)

Roche Registration GmbH; Treatment of spinal muscular atrophy

Day 30 discussion

Action: For discussion

Neurology

3.2.6. [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 30 discussion

Action: For discussion

Oncology

3.2.7. [Glucarpidase - EMEA-C-001391-PIP01-12](#)

Protherics Medicines Development BV; Treatment of methotrexate toxicity

Day 30 discussion

Action: For discussion

Oncology

3.2.8. [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 \(15-valent pneumococcal polysaccharide conjugate vaccine\) - EMEA-C1-002215-PIP01-17-M02](#)

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

Day 30 discussion

Action: For discussion

Vaccines

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

Novartis Europharm Limited; Treatment of rhabdomyosarcoma / Treatment of non-rhabdomyosarcoma soft tissue sarcoma / Treatment of Ewing sarcoma family of tumours

Day 30 discussion

Action: For discussion

Oncology

3.3. Discussions on Modification of an Agreed Paediatric Investigation Plan

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

Eli Lilly and Company Ltd; Benign prostatic hyperplasia (already approved in adults) / Pulmonary arterial hypertension (already approved in adults) / Treatment of persistent pulmonary hypertension of the newborn / Treatment of pulmonary arterial hypertension

Day 30 discussion

Action: For discussion

Cardiovascular Diseases

3.3.2. 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 30 discussion

Action: For discussion

Endocrinology-Gynaecology-Fertility-Metabolism

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

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

Day 30 discussion

Action: For discussion

Gastroenterology-Hepatology

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

Albireo AB; Progressive familial intrahepatic cholestasis (PFIC)

Day 30 discussion

Action: For discussion

Gastroenterology-Hepatology

3.3.5. 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 30 discussion

Action: For discussion

Gastroenterology-Hepatology

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

Pfizer Europe MA EEIG; Treatment of ulcerative colitis

Day 30 discussion

Action: For discussion

Gastroenterology-Hepatology

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

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

Day 30 discussion

Action: For discussion

Gastroenterology-Hepatology

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

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

Day 30 discussion

Action: For discussion

Immunology-Rheumatology-Transplantation

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

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

Day 30 discussion

Action: For discussion

3.3.10. 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 30 discussion

Action: For discussion

Infectious Diseases

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

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

Day 30 discussion

Action: For discussion

Infectious Diseases

3.3.12. 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 antiretroviral agents

Day 30 discussion

Action: For discussion

Infectious Diseases

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

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

Day 30 discussion

Action: For discussion

Infectious Diseases

3.3.14. Ibalizumab - EMEA-002311-PIP01-17-M01

Theratechnologies Europe Limited; Treatment of human immunodeficiency virus (HIV-1) infection / Ibalizumab, a CD4 domain 2-directed HIV-1 inhibitor, in combination with other antiretroviral(s), is indicated for the treatment of children and adolescents (aged 6 to less than 18 years) infected with HIV-1 resistant to at least 1 agent in 3 different classes

Day 30 discussion

Action: For discussion

Infectious Diseases

3.3.15. 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 30 discussion

Action: For discussion

Infectious Diseases

3.3.16. 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 30 discussion

Action: For discussion

Infectious Diseases

3.3.17. 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 30 discussion

Action: For discussion

Infectious Diseases

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

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

Day 30 discussion

Action: For discussion

Infectious Diseases

3.3.19. Rilpivirine (RPV) / dolutegravir (DTG) - EMEA-001750-PIP01-15-M03

ViiV Healthcare UK Limited; B24 Unspecified human immunodeficiency virus (HIV) disease / Treatment of human immunodeficiency virus type 1 (HIV-1) infection

Day 30 discussion

Action: For discussion

Infectious Diseases

3.3.20. [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 30 discussion

Action: For discussion

Infectious Diseases

3.3.21. [Hydrocortisone - EMEA-002305-PIP01-17-M01](#)

LABORATOIRE AGUETTANT; Bronchopulmonary dysplasia

Day 30 discussion

Action: For discussion

Neonatology - Paediatric Intensive Care

3.3.22. [Dopamine hydrochloride - EMEA-001105-PIP01-10-M05](#)

Brepco BioPharma; Treatment of hypotension in neonates including the extremely low gestational age newborn / Treatment of hypotension in infants and children / Treatment of hypotension in neonates including the extremely low gestational age newborn / Treatment of hypotension in infants and children

Day 30 discussion

Action: For discussion

Neonatology - Paediatric Intensive Care / Cardiovascular Diseases

3.3.23. [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 30 discussion

Action: For discussion

Neurology

3.3.24. [Lacosamide - EMEA-000402-PIP03-17-M04](#)

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

Day 30 discussion

Action: For discussion

Neurology

3.3.25. [Onasemnogene abeparvovec - Orphan - EMEA-002168-PIP01-17-M03](#)

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

Day 30 discussion

Action: For discussion

Neurology

3.3.26. [Ozanimod - EMEA-001710-PIP02-14-M05](#)

Celgene Europe B.V.; Treatment of multiple sclerosis

Day 30 discussion

Action: For discussion

Neurology

3.3.27. [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 30 discussion

Action: For discussion

Oncology

3.3.28. [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 30 discussion

Action: For discussion

Oncology

3.3.29. [Midostaurin - Orphan - EMEA-000780-PIP01-09-M05](#)

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

Day 30 discussion

Action: For discussion

Oncology

3.3.30. 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 30 discussion

Action: For discussion

Oncology

3.3.31. 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 as agreed by PDCO, in patients from 1 month to 18 years of age

Day 30 discussion

Action: For discussion

Oncology / Haematology-Hemostaseology

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

GenSight-Biologics; Leber hereditary optic neuropathy (LHON)

Day 30 discussion

Action: For discussion

Ophthalmology

3.3.33. 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 30 discussion

Action: For discussion

Other

3.3.34. Mometasone (furoate) / Indacaterol (acetate) - EMEA-001217-PIP01-11-M06

Novartis Europharm Limited; Treatment of asthma

Day 30 discussion

Action: For discussion

Pneumology - Allergology

3.3.35. 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 30 discussion

Action: For discussion

Pneumology - Allergology

3.3.36. Ferric citrate coordination complex - EMEA-001213-PIP02-12-M03

Akebia Therapeutics, Inc.; Treatment of hyperphosphataemia / Treatment of hyperphosphataemia in patients with chronic kidney disease (CKD)

Day 30 discussion

Action: For discussion

Uro-nephrology

3.3.37. 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 30 discussion

Action: For discussion

Uro-nephrology

3.3.38. 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 30 discussion

Action: For discussion

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 26 May 2020 for Nomination of Rapporteur and Peer reviewer

Action: For adoption

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

Action: For adoption

4.3. Nominations for other activities

Action: For adoption

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

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

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. Ranibizumab- EMEA-04-2020

Roche Products GmbH; All classes of medicinal products for treatment of age-related macular degeneration and diabetic macular oedema / Treatment of diabetic macular oedema / Treatment of neo vascular age-related macular degeneration

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

9.1.1. 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 on Herbal Medicinal Products (HMPC)

HMPC request for PDCO input

Action: For information

9.3. Coordination with EMA Working Parties/Working Groups/Drafting Groups

9.3.1. Non-clinical Working Group: D30 Products identified

PDCO member: Karen van Malderen

Action: For information

9.3.2. Formulation Working Group

PDCO member: Brian Aylward

Action: For information

9.3.3. Registry-based studies guideline - draft

Action: For information

9.4. Cooperation within the EU regulatory network

No item

9.5. Cooperation with International Regulators

No item

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. Business pipeline quarterly update report

Action: For information

10. Any other business

10.1.1. Covid-19 update

Action: For information

10.1.2. Debrief on the breakout session

Action: For information

10.1.3. Pharmaceutical strategy for Europe

Action: For information

11. Breakout sessions

11.1.1. Paediatric oncology

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

11.1.2. Neonatology

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

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/